UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
WASHINGTON, D.C. 20549

FORM 10-K

☒ **ANNUAL REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934**

For the fiscal year ended December 31, 2023

☐ **TRANSITION REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934**

For the transition period from ____ to _____

Commission File No. 001-36913

Zevra Therapeutics, Inc.
(Exact Name of Registrant as Specified in Its Charter)

Delaware **(State or Other Jurisdiction of Incorporation or Organization)**	**20-5894398** **(I.R.S. Employer Identification No.)**
1180 Celebration Boulevard, Suite 103, Celebration, FL 34747 **(Address of Principal Executive Offices and Zip Code)**	**(321) 939-3416** **(Registrant's Telephone Number, Including Area Code)**

Securities registered pursuant to Section 12(b) of the Act:

Title of Each Class	**Trading Symbol**	**Name of Each Exchange on Which Registered**
Common Stock, $0.0001 par value	**ZVRA**	**The Nasdaq Stock Market LLC** **(Nasdaq Global Select Market)**

Securities registered pursuant to Section 12(g) of the Act: None

Indicate by check mark if the Registrant is a well-known seasoned issuer, as defined in Rule 405 of the Securities Act Yes ☐ No ☒

Indicate by check mark if the Registrant is not required to file reports pursuant to Section 13 or Section 15(d) of the Act Yes ☐ No ☒

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Indicate by check mark whether the Registrant: (1) has filed all reports required to be filed by Section 13 or 15(d) of the Securities Exchange Act of 1934 during the preceding 12 months (or for such shorter period that the registrant was required to file such reports), and (2) has been subject to such filing requirements for the past 90 days. Yes ☒ No ☐

Indicate by check mark whether the Registrant has submitted electronically every Interactive Data File required to be submitted pursuant to Rule 405 of Regulation S-T during the preceding 12 months (or for such shorter period that the registrant was required to submit such files). Yes ☒ No ☐

Indicate by check mark whether the registrant is a large accelerated filer, an accelerated filer, a non-accelerated filer, smaller reporting company, or an emerging growth company. See the definitions of "large accelerated filer," "accelerated filer," "smaller reporting company," and "emerging growth company" in Rule 12b-2 of the Exchange Act.:

Large accelerated filer ☐ Accelerated filer ☐ Non-accelerated filer ☒ Smaller reporting company ☒ Emerging growth company ☐

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 13(a) of the Exchange Act. ☐

Indicate by check mark whether the registrant has filed a report on and attestation to its management's assessment of the effectiveness of its internal control over financial reporting under Section 404(b) of the Sarbanes-Oxley Act (15 U.S.C. 7262(b)) by the registered public accounting firm that prepared or issued its audit report. ☐

If securities are registered pursuant to Section 12(b) of the Act, indicate by check mark whether the financial statements of the registrant included in the filing reflect the correction of an error to previously issued financial statements. ☒

Indicate by check mark whether any of those error corrections are restatements that required a recovery analysis of incentive-based compensation received by any of the registrant's executive officers during the relevant recovery period pursuant to § 240.10D-1(b). ☐

Indicate by check mark whether the registrant is a shell company (as defined in Rule 12b-2 of the Exchange Act). Yes ☐ No ☒

The aggregate market value of the voting and non-voting common equity held by non-affiliates of the registrant as of June 30, 2023, the last business day of the registrant's most recently completed second fiscal quarter, was approximately $172.1 million, based upon the closing sales price for the registrant's common stock, as reported on the Nasdaq Global Select Market on June 30, 2023. The calculation of the aggregate market value of voting and non-voting common equity excludes 186,366 shares of common stock the registrant held by executive officers, directors and stockholders that the registrant concluded were affiliates of the registrant on that date. Exclusion of such shares should not be construed to indicate that any such person possesses the power, direct or indirect, to direct or cause the direction of management or policies of the registrant or that such person is controlled by or under common control with the registrant.

As of March 30, 2024, the registrant had 43,426,186 shares of common stock outstanding.

Documents Incorporated by Reference

Portions of the registrant's definitive proxy statement relating to its 2024 annual meeting of stockholders are incorporated by reference into Part III of this Annual Report on Form 10-K where indicated. Such proxy statement will be filed with the U.S. Securities and Exchange Commission within 120 days after the end of the fiscal year to which this report relates.

ZEVRA THERAPEUTICS, INC.
FORM 10-K

SPECIAL NOTE REGARDING FORWARD-LOOKING STATEMENTS

This Annual Report on Form 10-K, including the section entitled "Management's Discussion and Analysis of Financial Condition and Results of Operations," contains forward-looking statements regarding future events and our future results that are subject to the safe harbors created under the Securities Act of 1933, as amended, or the Securities Act, and the Securities Exchange Act of 1934, as amended, or the Exchange Act. Forward-looking statements relate to future events or our future financial performance. We generally identify forward-looking statements by terminology such as "may," "will," "would," "should," "expects," "plans," "anticipates," "could," "intends," "target," "projects," "contemplates," "believes," "estimates," "predicts," "assume," "intend," "potential," "continue" or other similar words or the negative of these terms. We have based these forward-looking statements largely on our current expectations about future events and financial trends that we believe may affect our business, financial condition and results of operations. The outcome of the events described in these forward-looking statements is subject to risks, uncertainties and other factors described in "Risk Factors" and elsewhere in this report. Accordingly, you should not place undue reliance upon these forward-looking statements. We cannot assure you that the events and circumstances reflected in the forward-looking statements will be achieved or occur, the timing of events and circumstances and actual results could differ materially from those anticipated in the forward-looking statements. Forward-looking statements contained in this report include, but are not limited to, statements about:

- *our ability to integrate Acer (as defined below) into our business successfully or realize the anticipated synergies and related benefits of the Merger (as defined below);*
- *the progress of, outcome or and timing of any regulatory approval for any of our product candidates and the expected amount or timing of any payment related thereto under any of our collaboration agreements;*
- *our ability to remediate the material weakness we have identified, the timing thereof, and the impact of the restatements described herein;*
- *our ability to continue as a going concern;*
- *the progress of, timing of and expected amount of expenses associated with our research, development and commercialization activities;*
- *our ability to raise additional funds on commercially reasonable terms, or at all, in order to support our continued operations;*
- *the sufficiency of our cash resources to fund our operating expenses and capital investment requirements for any period;*
- *the expected timing of our clinical trials for our product candidates and the availability of data and results of those trials;*
- *our expectations regarding federal, state and foreign regulatory requirements;*
- *the potential therapeutic benefits and effectiveness of our products and product candidates;*
- *the size and characteristics of the markets that may be addressed by our products and product candidates;*
- *our intention to seek to establish, and the potential benefits to us from, any strategic collaborations or partnerships for the development or sale of our products and product candidates, if approved;*
- *our expectations as to future financial performance, expense levels and liquidity sources;*
- *the timing of commercializing our products and product candidates, if approved;*
- *senior leadership and board member transitions and refreshments; and*
- *other factors discussed elsewhere in this report.*

The forward-looking statements made in this report relate only to events as of the date on which the statements are made. We have included important factors in the cautionary statements included in this report, particularly in the section entitled "Risk Factors" that we believe could cause actual results or events to differ materially from the forward-looking statements that we make. Our forward-looking statements do not reflect the potential impact of any future acquisitions, mergers, dispositions, joint ventures or investments we may make. Except as required by law, we do not assume any intent to update any forward-looking statements after the date on which the statement is made, whether as a result of new information, future events or circumstances or otherwise.

RISK FACTORS SUMMARY

The risk factors summarized below could materially harm our business, operating results, and/or financial condition, impair our future prospects, and/or cause the price of our common stock to decline. These risks are discussed more fully in the section titled "Risk Factors". Material risks that may affect our business, financial condition, results of operations, and trading price of our common stock include the following:

- *If commercialization of our approved products or our product candidates is not successful, or we experience significant delays in commercialization, our business will be harmed.*
- *If we are not able to obtain required regulatory approvals for our product candidates, or the approved labels are not sufficiently differentiated from other competing products, we will not be able to commercialize them and our ability to generate revenue or profits or to raise future capital could be limited.*
- *Our research and development activities are focused on discovering and developing transformational, patient-focused therapies for rare diseases with limited or no treatment options, which may never lead to additional marketable products.*
- *Arimoclomol is currently available to Niemann-Pick disease Type C patients in the United States. France, Germany, and other EU member states through our expanded access program, or EAP. The EAP is expected to remain in place until arimoclomol becomes commercially available in each of the current EAP markets. If the EAP is terminated prior to commercialization of arimoclomol, it will have a material adverse effect on our business, results of operations, cash flows, financial condition and/or prospects.*
- *The restatement of our consolidated financial statements for the year ended December 31, 2023, has subjected us to a number of additional risks and uncertainties.*
- *Management recently identified a material weakness in our internal control over financial reporting, which could have a significant adverse effect on our business and the price of our common stock.*
- *Our recurring operating losses and negative cash flows from operations raise substantial doubt about our ability to continue as a going concern.*
- *Clinical drug development involves a lengthy and expensive process, with an uncertain outcome. We may incur additional costs or experience delays in completing, or ultimately be unable to complete, the development and commercialization of our product candidates.*
- *We may need substantial additional funding to pursue our business objectives. If we are unable to raise capital when needed, we could be forced to delay, reduce or altogether cease our prodrug development programs or commercialization efforts or cease operations altogether.*
- *We have incurred significant recurring negative net operating losses since our inception. We expect to incur operating losses for the near future.*
- *If we are unable to obtain and maintain trade secret protection or patent protection for our technology, our approved products or our product candidates, or if the scope of the patent protection obtained is not sufficiently broad, our competitors could develop and commercialize technology and drugs similar or identical to ours, and our ability to successfully commercialize our technology, our approved products and our product candidates, if approved, may be impaired.*
- *If we attempt to rely on Section 505(b)(2) of the Federal Food, Drug and Cosmetic Act and the FDA does not conclude that our product candidates are sufficiently bioequivalent, or have comparable bioavailability, to approved drugs, or if the FDA does not allow us to pursue the 505(b)(2) NDA pathway as anticipated, the approval pathway for our product candidates will likely take significantly longer, cost significantly more and entail significantly greater complications and risks than anticipated, and the FDA may not ultimately approve our product candidates.*
- *The FDA may determine that any NDA we may submit under the 505(b)(2) regulatory pathway for any of our product candidates in the future is not sufficiently complete to permit a substantive review.*
- *We have entered into collaborations with Commave Therapeutics, S.A., or Commave, to develop, manufacture and commercialize AZSTARYS worldwide. In addition, we may seek collaborations with third parties for the development, manufacturing or commercialization of our other product candidates, or in other territories. If those collaborations are not successful, we may not be able to capitalize on the market potential of our approved products or other product candidates, if approved.*
- *The trading price of the shares of our common stock is likely to be volatile, and purchasers of our common stock could incur substantial losses.*
- *Our future success depends on our ability to retain key executives and to attract, retain and motivate qualified personnel.*
- *We could be negatively affected as a result of the actions of activist stockholders, which could be disruptive and costly and may conflict with or disrupt the strategic direction of our business.*
- *Adverse developments affecting the financial services industry, including events or concerns involving liquidity, defaults, or non-performance by financial institutions, could adversely affect our business, financial condition or results of operations.*

NOTE REGARDING COMPANY REFERENCE

Unless the context otherwise requires, we use the terms "Zevra," "Company," "we," "us" and "our" in this Annual Report on Form 10-K to refer to Zevra Therapeutics, Inc. We have proprietary rights to a number of trademarks used in this Annual Report on Form 10-K that are important to our business, including LAT®, OLPRUVA® and its related logo, and the Zevra logo. All other trademarks, trade names and service marks appearing in this Annual Report on Form 10-K are the property of their respective owners. Solely for convenience, the trademarks and trade names in this Annual Report on Form 10-K are referred to without the ® and ™ symbols, but such references should not be construed as any indicator that their respective owners will not assert, to the fullest extent under applicable law, their rights thereto.

On August 30, 2023, the Company and Aspen Z Merger Sub, Inc., a wholly-owned subsidiary of Zevra ("Merger Sub") entered into an Agreement and Plan of Merger (the "Merger Agreement") with Acer Therapeutics, Inc. ("Acer"). On November 17, 2023 (the "Closing Date"), we completed the acquisition of Acer. Pursuant to the Merger Agreement, on the Closing Date, Merger Sub was merged with an into Acer (the "Merger"), with Acer continuing as the surviving entity and as a wholly-owned subsidiary of Zevra.

NOTE REGARDING MARKET AND INDUSTRY DATA

This Annual Report on Form 10-K contains statistical data, estimates and forecasts that are based on independent industry publications or other publicly available information, as well as other information based on our internal sources. While we believe the industry and market data included in this Annual Report on Form 10-K is reliable and is based on reasonable assumptions, this data involves many assumptions and limitations, and you are cautioned not to give undue weight to these estimates. We have not independently verified the accuracy or completeness of the data contained in these industry publications and other publicly available information. The industry in which we operate is subject to a high degree of uncertainty and risk due to a variety of factors, including those described in the section titled "Risk Factors" and "Special Note Regarding Forward-Looking Statements" included in this Annual Report on Form 10-K.

EXPLANATORY NOTE

Restatement of Consolidated Financial Results

On March 25, 2024, the Audit Committee (the "Audit Committee") of our Board of Directors, after discussion with senior management and the Company's independent registered public accountants, concluded that our previously issued audited consolidated financial statements as of and for the fiscal years ended December 31, 2022 and December 31, 2021, included in the Company's Annual Report on Form 10-K for the fiscal year ended December 31, 2022, (collectively, the "Prior Financial Statements") should no longer be relied upon. In connection with the preparation of this Annual Report on Form 10-K for the fiscal year ended December 31, 2023, the Audit Committee concluded that, in prior years it had not appropriately accounted for certain common stock warrants as liabilities. These errors led to understatements of derivative and warrant liability and additional paid-in capital and fluctuations in fair value adjustment related to derivative and warrant liability during the impacted periods.

In addition, the Audit Committee concluded that the previously disclosed errors led to misstatements of fair value adjustment related to derivative and warrant liability, derivative and warran liability, additional paid-in capital, and accumulated deficit that were previously disclosed in the unaudited condensed consolidated balance sheets and statements of operations included in our Quarterly Reports on Form 10-Q for the quarterly periods ended March 31, 2022, June 30, 2022, September 30, 2022, March 31, 2023, June 30, 2023, and September 30, 2023 (collectively, the "Prior Interim Financial Statements"). On March 25, 2024, the Audit Committee, after discussion with senior management and the Company's independent registered public accountants concluded that the Prior Interim Financial Statements should no longer be relied upon.

As a result, we are restating our consolidated financial statements for the year ended December 31, 2022, and the Prior Interim Financial Statements in this Annual Report on Form 10-K. In addition, the following items of this Annual Report on Form 10-K include restated financial data: (i) Part II, Item 7—Management's Discussion and Analysis of Financial Condition and Results of Operations; (ii) Part II, Item 8—Financial Statements and Supplementary Data (which incorporated by reference the information from Part IV, Item 15—Exhibits and Financial Statemen Schedules) and (iii) Part IV, Item 15—Exhibits and Financial Statement Schedules. Note C to our consolidated financial statements sets forth, in a comparative presentation, the previously reported, restatement adjustments and restated amounts for those line items in the relevant periods affected by the restatement. This Annual Report on Form 10-K also includes disclosure regarding the impact of the restatement on the effectiveness of the Company's internal control over financial reporting and disclosure controls and procedures in Part II, Item 9A.— Controls and Procedures.

We have not amended our previously-filed Annual Reports on Form 10-K and Quarterly Reports on Form 10-Q for the periods noted above. Instead, such financial statements are superseded by the audited consolidated financial statements for the year ended December 31, 2023, contained in this Annual Report on Form 10-K, which give effect to the restatements noted above

PART I

ITEM 1. BUSINESS.

Overview

We are a rare disease company combining science, data, and patient needs to create transformational therapies for diseases with limited or no treatment options. Our mission is to bring life-changing therapeutics to people living with rare diseases. With unique, data-driven development and commercialization strategies, the Company is overcoming complex drug development challenges to make new therapies available to the rare disease community. We have a diverse portfolio of products and product candidates, which includes preclinical development programs, clinical stage pipeline and commercial stage assets. Our team has specialized expertise and a track record of success in advancing promising therapies that face complex clinical and regulatory challenges with an approach that balances science and data with patient need.

Following the U.S. approval of AZSTARYS® (further described below) in March 2021, we undertook a strategic process to evaluate how to leverage and potentially augment the Company's existing capabilities while also considering where to invest in our pipeline to generate long-term shareholder value. With a track record of drug development success leading to approvals for products which had either difficult pathways to approval or where approvals were won following a complete response letter ("CRL") from the U.S. Food and Drug Administration ("FDA"), the Company determined to focus its expertise on rare disease indications, as well as seeking value-creating opportunities by building and directly commercializing product candidates in lieu of an out-licensing model. We are executing on this balanced approach by building a culture that is patient-focused and driven by our commitment to developing and making available therapies which address the myriad unmet needs within the rare disease community.

As part of our commitment to serving the rare disease community, in February 2023, we changed our name to Zevra Therapeutics, Inc. Our name, Zevra, is the Greek word for zebra, which is the internationally recognized symbol for rare disease. This name reflects our intense focus and dedication to developing transformational, patient-focused therapies for rare diseases with limited or no treatment options available, or treatment areas with significant unmet needs.

In order to accomplish our mission, we are seeking to further expand our pipeline through both internal development and through our business development activities to collaborate, partner, and potentially acquire additional assets. We intend to target assets that will allow us to leverage the expertise and infrastructure that we have built in order to mitigate risk and enhance our probability of success. In addition, we may consider external opportunities within neurology and neurodegenerative diseases, psychiatric disorders, and other rare diseases, along with adjacent or related therapeutic categories. If we are successful, expanding our pipeline could be accretive to our value proposition and has the potential to create incremental long-term value.

In May 2022, we purchased all of the assets and operations of Orphazyme A/S related to arimoclomol, settled all of Orphazyme's actual outstanding liabilities to its creditors with a cash payment of $12.8 million, and agreed to assume an estimated reserve liability of $5.2 million related to revenue generated from Orphazyme's Expanded Access Program in France (the "Arimoclomol EAP").

On November 17, 2023, Zevra completed the acquisition of Acer. Pursuant to the Merger Agreement, Acer continues as a wholly-owned subsidiary of Zevra. The Merger included the acquisition of OLPRUVA® (sodium phenylbutyrate) for oral suspension, which was approved by the U.S. Food and Drug Administration (FDA) on December 27, 2022, for the treatment of urea cycle disorders ("UCDs"). Acer also had a pipeline of investigational product candidates, including celiprolol for the treatment of vascular Ehlers-Danlos syndrome ("vEDS") in, patients with a confirmed type III collagen (COL3A1) mutation. At the effective time of the Merger (the "Effective Time"), each share of common stock of Acer, par value $0.0001 per share, issued and outstanding immediately prior to the Effective Time (excluding cancelled shares and any shares held by holders who have exercised their appraisal rights) were converted into the right to receive (i) 0.1210 fully paid and non-assessable shares of common stock of Zevra, par value $0.0001 per share, and (ii) one non-transferable contingent value right ("CVR") issued by Zevra, which represents the right to receive one or more contingent payments up to an additional $76.0 million upon the achievement, if any, of certain commercial and regulatory milestones for Acer's OLPRUVA and celiprolol products within specified time periods. Certain additional cash payments are also possible pursuant to the CVRs with respect to milestones involving Acer's early-stage program ACER-2820 (emetine).

Our recurring operating losses and negative cash flows from operations raise substantial doubt about our ability to continue as a going concern. We have no current source of revenues to sustain our present activities. Accordingly, our ability to continue as a going concern will require us to obtain additional financing to fund our operations. The perception of our inability to continue as a going concern may make it more difficult for us to obtain financing for the continuation of our operations and could result in the loss of confidence by investors, suppliers and employees. Adequate additional financing may not be available to us on acceptable terms, or at all. To the extent that we raise additional capital through the sale of equity or debt, the terms of these securities may restrict our ability to operate. If we raise additional funds through collaborations, strategic alliances or marketing, distribution or licensing arrangements with third parties, we may be required to relinquish valuable rights. If we are unable to raise capital when needed or on attractive terms, we could be forced to delay, reduce or altogether cease our research and development programs or future commercialization efforts.

Our Product Candidates and Approved Products

We have built a diverse portfolio of products and product candidates through a combination of internal development and strategic investments through acquisition. For example, we have employed our proprietary Ligand Activated Technology ("LAT") platform to develop approved products (e.g., AZSTARYS), and clinical development candidates (KP1077IH and KP1077N). Through our business development efforts, we have added a commercial product (OLPRUVA), and clinical development candidates (arimoclomol, celiprolol). We furthermore have a variety of product candidates and compounds that are early-stage, pre-clinical and clinical-stage designed to address a variety of rare diseases and other indications.

Currently active commercial products and development assets are summarized in the table below:

Active Zevra Commercial and Development Assets

Parent Drug	Indication	Product / Candidate	Development Status	Next Milestone(s)
Sodium phenylbutyrate	Urea Cycle Disorders (UCD)	OLPRUVA	FDA Approved	Tracking Commercial Progress
Arimoclomol	Niemann Pick disease type C (NPC)	Arimoclomol	Pending FDA Review	PDUFA target date September 21, 2024
Celiprolol	Vascular Ehlers Danlos Syndrome (vEDS)	Celiprolol	Clinical - Phase 1/2	Phase 3 ongoing
Serdexmethylphenidate	Idiopathic Hypersomnia (IH)	KP1077IH	Clinical - Phase 2	Evaluation of potential Phase 3 Trial
Serdexmethylphenidate	Narcolepsy	KP1077N	Clinical - Phase 1/2	Evaluation of potential Phase 3 Trial
Serdexmethylphenidate and dexmethylphenidate	Attention Deficit and Hyperactivity Disorder (ADHD)	AZSTARYS	FDA Approved and Partnered	Collecting royalties and milestones

These anticipated milestones are based on information currently available to us. Our current plans and expectations are subject to a number of uncertainties, risks and other important factors that could materially impact our plans, including risks which are not solely within our control. See Part I, Item 1A. “Risk Factors” in this Annual Report on Form 10-K.

OLPRUVA

OLPRUVA (sodium phenylbutyrate) for oral suspension is approved in the U.S. as adjunctive therapy to standard of care, which includes dietary management, for the chronic management of urea cycle disorders (UCDs) involving deficiencies of carbamylphosphate synthetase (CPS), ornithine transcarbamylase (OTC), orargininosuccinic acid synthetase (AS). OLPRUVA is a proprietary and novel formulation of sodium phenylbutyrate powder, packaged in pre-measured single-dose envelopes, that has shown bioequivalence to existing sodium phenylbutyrate powder but with a pH-sensitive polymer coating that is designed to minimize dissolution of the coating for up to five minutes after preparation.

UCDs are a group of rare, genetic disorders that can cause harmful ammonia to build up in the blood, potentially resulting in brain damage and neurocognitive impairments, if ammonia levels are not controlled. Any increase in ammonia over time is serious. Therefore, it is important to adhere to any dietary protein restrictions and have alternative medication options to help control ammonia levels. Approximately 1 in 100,000 people have UCD, and there are an estimated 800 patients who are actively treated in the U.S. While there are therapies currently approved for the treatment of UCDs - specifically RAVICTI®, marketed by Amgen, Inc. (formerly Horizon Therapeutics) and PHEBURANE®, marketed by Medunik USA - there remain unmet needs for this community of patients. OLPRUVA offers benefits over other UCD treatments by eliminating issues with palatability, offering improved portability with its single-dose envelopes, and it comes in a dosage that personalized to the patient based on weight.

To commercialize OLPRUVA for oral suspension in the U.S. we have built in-house capabilities including rare disease sales specialists who are working with prescribing clinicians and healthcare providers, as well as marketing, patient reimbursement services, market access and contracting, patient advocacy, sales, and medical affairs teams. This team was hired and trained between the end of 2023 and early January, with full launch effective January 29, 2024. We have also made arrangements with third parties to provide these additional services such as distribution and specialty pharmacy offerings.

During the quarter ended December 31, 2023, and following the completion of our mergers with Acer Therapeutics, Inc. on November 17, 2023 (the “Merger”), we began generating revenue from the sale of OLPRUVA in the U.S. For additional information regarding the Merger, see Note R of our audited consolidated financial statements included elsewhere in this Annual Report on Form 10-K. Zevra has a partnership with Relief Therapeutics who has rights to commercialize OLPRUVA in various EU countries, if approved. In addition, Zevra pays royalties of 10% of net sales plus milestones to Relief Therapeutics based on US net sales.

OLPRUVA summary:

- ***OLPRUVA is available in the U.S for the treatment of UCD.*** OLPRUVA is an adjunctive therapy for long-term management of adults and children weighing 20kg or greater with UCD from deficiencies of CPS, OTC, or AS.

- ***OLPRUVA is differentiated from currently available forms of phenylbutyrate.*** OLPRUVA is formulated to improve palatability while providing patients with a portable and discrete pre-measured dose.

- ***Zevra has assembled a team to support OLPRUVA and additional future commercial products.*** We have established an efficient commercial team which is designed to fully service the patients and prescribers within the rare disease indications we are pursuing.

Arimoclomol

Arimoclomol is our product candidate being developed for the treatment of Niemann-Pick disease type C (NPC), an ultra-rare neurodegenerative lysosomal storage disorder (LSD). Arimoclomol is an orally delivered, first in-class investigational product candidate which has been granted orphan drug designation, Fast-Track designation, Breakthrough Therapy designation and rare pediatric disease designation for the treatment of NPC by the FDA, and orphan medicinal product designation for the treatment of NPC by the European Commission. The arimoclomol New Drug Application (NDA) was submitted to the FDA on December 21, 2023, and is currently undergoing review by the FDA. The FDA has assigned a PDUFA date of September 21, 2024. We believe that, if approved by the FDA, arimoclomol will be eligible to receive a Rare Pediatric Disease Priority Review Voucher (PRV), which is transferrable.

As an LSD, NPC is characterized by an inability of the body to transport cholesterol and lipids inside of cells. Symptoms of NPC include a progressive impairment of mobility, cognition, speech, and swallowing, often culminating in premature death. The incidence of NPC is estimated to be one in 100,000 to 130,000 live births. We estimate that there are approximately 1,800 individuals with NPC in the US and Europe, of these, approximately 300 have been diagnosed in the U.S. However, diagnostic challenges may affect the number of potential patients, and we believe that the availability of treatment options could increase awareness of the disease and assist in more accurately identifying patients. Effective therapies to treat NPC are desperately needed, and for this reason, arimoclomol is currently being made available to NPC patients in the United States, France, Germany, and other EU member states under various expanded access programs ("EAPs").

On September 16, 2020, the previous sponsor of the arimoclomol program, Orphazyme, submitted an NDA to the FDA, seeking approval for arimoclomol to treat NPC. In June 2021, the FDA issued a complete response letter ("CRL"), meaning it determined that it could not approve the NDA in its present form.

Zevra acquired the assets of Orphazyme A/S (Orphazyme) in May 2022, and took over the responsibility for arimoclomol, including the preparation and resubmission of the NDA designed to respond to the FDA's specific deficiencies identified in the CRL and feedback in subsequent meetings between the FDA and Orphazyme. Since that time, we have worked diligently to characterize the meaningful evidence of safety and efficacy of arimoclomol for its intended use and the substantial data generated since the CRL, including the recently completed four-year open-label safety trial, an interim analysis of which was presented at the 19th World*Symposium*™ in February 2023. Upon fulfilling the randomized double-blinded portion of the Phase 2/3 clinical trial, both placebo- and arimoclomol-treated patients were given the option to continue into the four-year (48 month) open-label-extension ("OLE"), phase of the study with arimoclomol treatment provided in addition to their current standard of care. We believe that the results from this analysis, based on up to four years of continuous treatment, suggest that arimoclomol may reduce the long-term progression of NPC.

In preparation of the arimoclomol NDA resubmission, we completed a meeting with the FDA in August 2023, receiving feedback that was used to finalize the NDA submission. The updated NDA package for arimoclomol was resubmitted to the FDA in December 2023. Zevra believes it has addressed the issues previously raised by the FDA in the 2021 CRL. Zevra has conducted additional studies to support the potential mechanism of action of arimoclomol. Additionally, new data was included in the resubmission as supportive evidence from multiple non-clinical studies, natural history comparisons, real-world data generated from the ongoing early access programs in the U.S. and the European Union, as well as data from the four-year open-label extension of the Phase 2/3 clinical trial (NCT02612129).

In January 2024, the FDA acknowledged receipt of the resubmission and, under the Prescription Drug User Fee Act ("PDUFA"), deemed the arimoclomol NDA resubmission to be a Class II complete response which has a six-month review period from the date of resubmission. On March 4, 2024, we announced that the FDA had extended the review period for the NDA for arimoclomol and set a new PDUFA date of September 21, 2024. The FDA also re-affirmed its intent to present the resubmission for discussion at an advisory committee meeting to be scheduled.

Zevra holds the global rights for arimoclomol. We are evaluating the possibility of seeking regulatory approval and commercialization outside of the US.

Arimoclomol summary:

- ***Currently, no approved treatments for NPC in the U.S.*** There are no currently approved products in the U.S. to treat the underlying disease of NPC and we believe, if approved, arimoclomol could be considered a foundational therapy for patients in the U.S.

- ***Designed to address disease progression.*** Arimoclomol is designed to address the symptoms of NPC by slowing the progression of the disease itself, rather than serving as a symptomatic treatment only. The Phase 2/3 trial data for arimoclomol in NPC demonstrated reduced disease progression, and long-term data from the 4-year OLE of the Phase 2/3 trial suggest improved outcomes vs. historical controls.

- ***Ease of flexible administration as an oral treatment.*** Arimoclomol is administered as an oral capsule that can be swallowed whole, opened and contents mixed with foods or liquids, or delivered through a feeding tube.

- ***Extensive clinical experience with favorable safety data.*** No significant safety findings have been reported with more than 600 patients treated in various clinical trials and through our expanded access programs.

- ***Advantageous regulatory designations.*** Arimoclomol has been granted orphan drug designation, Fast Track designation, and Breakthrough Therapy designation for the treatment of NPC. If approved for the treatment of NPC, we believe arimoclomol will be eligible to receive a Pediatric Rare Disease Priority Review Voucher ("PRV").

Celiprolol

The Merger with Acer included the acquisition of celiprolol. We are advancing celiprolol as an investigational product candidate for the treatment of vascular Ehlers-Danlos syndrome (vEDS) in patients with a confirmed type III collagen (*COL3A1*) mutation. Celiprolol is a selective adrenergic modulator (SAM) and, if we receive the first approval in the U.S. for celiprolol, we believe it would be deemed a new chemical entity ("NCE") in the U.S. Celiprolol is currently approved in the European Union for the treatment of hypertension and angina.

Ehlers-Danlos syndrome ("EDS") is an inherited disorder caused by mutations in the genes responsible for the structure, production, or processing of collagen, an important component of the connective tissues in the human body, or proteins that interact with collagen. vEDS causes abnormal fragility in blood vessels, which can give rise to aneurysms, abnormal connections between blood vessels known as arteriovenous fistulas, arterial dissections, and spontaneous vascular ruptures, all of which can be potentially life-threatening. Gastrointestinal and uterine fragility or rupture also commonly occur in vEDS patients. Spontaneous arterial rupture has a peak incidence in the third or fourth decade of life in vEDS patients but may occur earlier and is the most common cause of sudden death in vEDS patients. Arterial rupture or dissection events occur in about 25% of patients before the age of 20 but increase to roughly 90% of patients by the age of 40. The median survival age of vEDS patients in the U.S. is 51 years, with arterial rupture being the most common cause of sudden death. Pregnancy-related complications also occur in women with vEDS and include arterial dissection or rupture, uterine rupture, hemorrhage, premature rupture of membranes, lacerations, and complications during and after surgery. The incidence of vEDS is estimated to be one in 50,000 to 200,000 people. There are approximately 7,500 diagnosed patients in the U.S.

Currently, there are no approved therapies anywhere in the world for vEDS. However, celiprolol, prescribed off label, has become the standard of care therapy for vEDS in some Europe an countries. Medical intervention for vEDS focuses on surgery, symptomatic treatment, genetic counseling, and prophylactic measures, such as avoiding intense physical activity, scuba diving, and violent sports. Arterial, digestive, or uterine complications in vEDS patients typically require immediate hospitalization, observation in an intensive care unit, and sometimes surgery. Pregnant women with vEDS are considered to be at risk and receive special care. While vEDS patients are encouraged to take steps to minimize the chances of an arterial rupture or dissection, there are no pharmacologic options to reduce the likelihood of such an event, and accordingly current treatments for vEDS focus on the repair of arterial ruptures or dissection. Therefore, patients must adopt a "watch and wait" approach following any confirmed diagnosis. Unfortunately, many of these arterial events have high mortality associated with them, and thus, a pharmacologic intervention that reduces the rate of events would be clinically meaningful.

Celiprolol has not been approved for any indication in the U.S. In the past, an NDA for celiprolol for the treatment for hypertension was submitted to the FDA by Rorer (subsequently acquired by Aventis Pharma SA (Aventis)) in June 1987, but was subsequently withdrawn prior to completion of the FDA review and therefore never approved. We have obtained the exclusive right in North and South America from Aventis to reference the celiprolol data included in the marketing authorization application dossier filed with and approved by the UK Medicines and Healthcare Products Regulatory Agency ("MHRA"). In addition, our wholly-owned subsidiary, Acer Therapeutics, Inc. ("Acer") has licensed exclusive worldwide rights to the data from the Phase 3 clinical trial known as the BBEST trial which was sponsored by L'Assistance Publique Hôpitaux de Paris ("AP-HP").

Celiprolol received orphan drug designation from the FDA for the treatment of vEDS in 2015. In October 2018, a new celiprolol NDA was submitted to the FDA by Acer based on data obtained from the BBEST trial and was subsequently accepted by the FDA in October 2018 with priority review status. Following FDA review, Acer received a CRL from the FDA stating that it will be necessary to conduct an adequate and well-controlled trial to determine whether celiprolol reduces the risk of clinical events in patients with vEDS. Subsequently, Acer appealed the FDA decision, and while the FDA denied the appeal, it described possible paths forward toward approval. In a May 2021 Type B meeting with the FDA, Acer discussed the conduct of an U.S.-based prospective, randomized, double-blind, placebo-controlled, decentralized clinical trial in patients with *COL3A1* positive vEDS, and sought the FDA's opinion on various proposed design features of the study.

Based on FDA's feedback during the Type B meeting, we adopted a decentralized (virtual) event-based clinical trial design and use of an independent centralized adjudication committee with a primary endpoint based on clinical events associated with disease outcome. In April 2022, the FDA granted celiprolol Breakthrough Therapy designation ("BTD") in the U.S. for the treatment of patients with COL3A1-positive vEDS.

In July 2022, Acer initiated enrollment in a phase 3 clinical trial designed based on the discussions from the May 2021 Type B meeting with the FDA, also known as the DiSCOVER trial. The DiSCOVER trial intends to enroll 150 vEDS patients, with 100 patients receiving celiprolol and 50 patients receiving placebo. The first patient was dosed in November 2022 and the trial is currently enrolling.

Celiprolol summary:

- ***Currently, no approved treatments for vEDS in the U.S.*** There are currently no approved treatments of vEDS in the U.S. and we believe that celiprolol, if approved, could be a significant innovation in the treatment of vEDS in the U.S. where current treatment options are focused primarily on surgical intervention.

- ***Unique pharmacological profile.*** Mechanism of action in vEDS patients is thought to be through vascular dilatation and smooth muscle relaxation, the effect of which is to reduce the mechanical stress on collagen fibers in the arterial wall, and thereby potentially less incidence of vascular ruptures.

- ***Evidence of efficacy in the E.U and extensive clinical experience from multiple trials.*** Celiprolol has become the primary treatment for vEDS patients in several European countries. BBEST Clinical Trial data showed 76% reduction in risk of arterial events observed in COLA3A1+ subpopulation, with additional data from a long-term observational study in France.

- ***Regulatory designations.*** Celiprolol for vEDS would be considered an NCE in the U.S. and has been granted Orphan Drug designation and Breakthrough Therapy designation.

- ***Solid patent protection through 2038.*** Celiprolol is generally protected by U.S. patents that will expire, after utilizing all appropriate patent term adjustments but excluding possible term extensions, in 2038.

KP1077

KP1077 is being developed for the treatment of IH and narcolepsy. IH is a rare neurological sleep disorder affecting approximately 37,000 patients in the United States. The cardinal feature of IH is excessive daytime sleepiness ("EDS"), characterized by daytime lapses into sleep, or an irrepressible need to sleep that persists even with adequate or prolonged nighttime sleep. Additionally, those with IH have extreme difficulty waking, otherwise known as “sleep inertia,” suffer from severe and debilitating brain fog, and may fall asleep unintentionally or at inappropriate times, also known as narcolepsy. These symptoms often further lead to reported memory problems, difficulty maintaining focus, and depression.

There is currently only one approved product for the treatment of IH, XYWAV®, developed by Jazz Pharmaceuticals. A second product, WAKIX®, developed by Harmony Biosciences and originally approved for the treatment of EDS or cataplexy in adult patients with narcolepsy, but in October 2023, Harmony announced that the difference in outcome for EDS when comparing WAKIX and placebo in its Phase 3 trial with IH patients did not reach statistical significance. Prescribers also utilize narcolepsy medications and various stimulant products “off-label” to treat IH symptoms, with methylphenidate, a stimulant which has been classified by the DEA as a Schedule II controlled substance, being one of the most commonly used stimulants for treating IH. While each of these medications can help to address certain IH symptoms, there are also potential shortcomings, including dosing inconvenience, serious adverse events, such as elevated blood pressure and heart rate, and significant drug-to-drug interactions ("DDIs"), including with medications used to manage contraception and depression. In addition, patients have indicated that the effectiveness of their current medication was poor.

Narcolepsy is a rare, chronic, debilitating neurologic disorder of sleep-wake state instability that impacts up to 200,000 Americans and is primarily characterized by EDS and cataplexy (sudden loss of muscle tone while a person is awake) along with other manifestations of rapid eye movement ("REM"), sleep dysregulation, which intrude into wakefulness. In most patients, narcolepsy is caused by the loss of hypocretin, a neuropeptide in the brain that supports sleep-wake state stability. Typical symptom onset occurs in adolescence or young adulthood, but it can take up to a decade to be properly diagnosed. Although there are several approved medications for narcolepsy, we believe a treatment option based on serdexmethylphenidate (“SDX"), our proprietary prodrug of d-methylphenidate (“d-MPH”) which has previously been classified as a Schedule IV controlled substance, with superior exposure/duration characteristics and low abuse potential may be beneficial.

We reported top-line data from a Phase 1 proof-of-concept study of SDX in the fourth quarter of 2021 and final data for the Phase 1 proof-of-concept study of SDX in the first quarter of 2022. The proof-of-concept study was a dose-escalation study to evaluate the pharmacokinetics, pharmacodynamic stimulant effects, and safety of single oral doses of SDX in subjects with a history of high-dose stimulant use. In the trial, 240 mg and 360 mg doses of SDX were observed to be well-tolerated and produced d-MPH exposure that appeared to increase proportionally with dose. Mean d-MPH plasma concentrations showed a gradual increase after SDX administration, reaching a broad peak from eight to twelve hours post-dose, followed by a shallow decline thereafter. Increased wakefulness, alertness, hypervigilance, and insomnia effects were reported by study participants, which we believe suggests that SDX produced targeted pharmacodynamic effects that have the potential to benefit patients with IH and other sleep disorders. In November 2022, we announced that the FDA has granted the orphan drug designation to SDX for the treatment of IH.

In January 2022, we announced that we had selected KP1077 for the treatment of IH and narcolepsy as our lead clinical development candidate. KP1077 utilizes SDX, our prodrug of d-MPH, as its API. During the first quarter of 2022, we initiated a Phase 1 clinical trial comparing the cardiovascular safety of SDX to immediate-release and long-acting formulations of RITALIN®, a commonly prescribed CNS stimulant. In September 2022, we announced topline data from our exploratory Phase 1 clinical trial, which showed the potential for higher dose formulations of SDX to be safe and well tolerated while avoiding the potential for greater cardiovascular safety risk compared to immediate-release and long-acting formulations of Ritalin.

Based on the data, in December 2022, we announced the initiation of a double-blind, placebo-controlled, randomized-withdrawal, dose-optimizing, multi-center Phase 2 clinical trial evaluating the efficacy and safety of KP1077 for the treatment of IH. The trial concluded in March 2024 and provided meaningful information of the optimal dose and dosing regimen to inform Phase 3 trial design.

We enrolled 48 adult patients with IH in more than 30 centers in the United States. Part 1 of the trial consisted of a five-week open-label titration phase during which patients were optimized to one of four doses of SDX (80, 160, 240, or 320 mg/day). Part 2 of the trial entailed a two-week randomized, double-blind, withdrawal phase, during which two-thirds of the trial participants will continue to receive their optimized dose while the remaining one-third will receive placebo. Participants were further assigned into two evenly divided cohorts. The first cohort received a single daily dose just before bedtime, and the second cohort received half the daily dose shortly after awakening and half the daily dose prior to bedtime.

Clinically meaningful improvements were observed across all studied endpoints. The trial was not powered for statistical significance, and this was not the primary endpoint. The exploratory endpoints of sleep inertia and brain fog performed in-line with expectations and were stable when compared across a variety of other endpoints. Symptom improvements in patients receiving KP1077 were similar after both once-per-day, and twice-per-day dosing.

In the Phase 2 trial, KP1077 was observed to be well-tolerated at all dose levels and both dosing regimens, with adverse events that are typical for stimulants and mostly mild in severity. These results are consistent with data from the Phase 1 trial with serdexmethylphenidate (SDX) that indicated no greater cardiovascular safety risk despite higher overall exposure levels when compared to both immediate and long-acting methylphenidate products currently used off-label for the treatment of IH.

In the second quarter of 2023, we initiated a Phase 1 clinical trial in healthy volunteers to assess proposed dosing regimen for the narcolepsy indication. This study was completed in September 2023. By leveraging the data from the IH program, Zevra is evaluating the potential to initiate a Phase 3 trial in narcolepsy.

KP1077 is subject to a right of first negotiation upon completion of a proof-of-concept study in favor of Commave, under the terms of the AZSTARYS License Agreement, but is not currently licensed to Commave, thereunder.

KP1077 Summary:

- ***Dosing flexibility.*** Designed to be delivered in either one or two doses daily, which is designed to address the two primary issues associated with IH: (i) nighttime dose would address sleep inertia, and (ii) morning dose would address daytime brain fog.

- ***No drug-to-drug interactions.*** We have not observed drug-to-drug interactions in clinical drug-drug interaction studies.

- ***Potential for reduced abuse potential as a Schedule IV controlled substance.*** All other methylphenidate-based products have been designated as Schedule II controlled substances, which indicates stricter control over the prescribing and use of such products. KP1077 is based on SDX, which has been designated a Schedule IV controlled substance.

- ***No currently approved generic equivalent product.*** KP1077 contains SDX, our proprietary prodrug of d-methylphenidate, also known as the new chemical name, serdexmethylphenidate, by the U.S. Adopted Names Council of the American Medical Association (“USAN”), which means that there may be no generic equivalent product for KP1077 in most states, making drug-equivalent substitution potentially difficult at the pharmacy.

- ***Orphan drug designation.*** Because small size of the IH patient population, the FDA has granted KP1077 orphan drug designation for the treatment of IH. We believe KP1077 may potentially be eligible for fast-track and breakthrough therapy designation, which may provide various regulatory benefits for the development program.

AZSTARYS (Partnered product)

AZSTARYS contains dexmethylphenidate (d-MPH) and our prodrug of dexmethylphenidate, serdexmethylphenidate (SDX). On March 2, 2021, the FDA approved AZSTARYS as a once-daily treatment for attention deficit hyperactivity disorder (ADHD), in patients age six years and older. AZSTARYS is currently being marketed in the U.S. under our September 2019 collaboration and license agreement, or the AZSTARYS License Agreement, with Commave Therapeutics SA (formerly known as Boston Pharmaceutical S.A.) ("Commave"), an affiliate of Gurnet Point Capital, L.P. Under the AZSTARYS License Agreement, we granted to Commave an exclusive, worldwide license, to develop, manufacture, and commercialize AZSTARYS and any of our product candidates containing SDX and used to treat ADHD or any other central nervous system ("CNS") disease.

Commave has tasked Corium, Inc. ("Corium"), another affiliate of Gurnet Point Capital, L.P., to lead all commercialization activities for AZSTARYS in the U.S. Corium commercially launched AZSTARYS in the U.S. during the third quarter of 2021. In December 2021, Commave entered into a sublicense of commercialization rights for AZSTARYS in greater China to Shanghai Ark Biopharmaceutical Ltd.

Pursuant to the AZSTARYS License Agreement, Commave agreed to pay up to $63.0 million in milestone payments upon the occurrence of specified regulatory milestones related to the AZSTARYS, including FDA approval and specified conditions with respect to the final approval label. In addition, Corium agreed to make additional payments upon the achievement of specified U.S. sales milestones of up to $420 million in the aggregate. Further, Commave will pay us quarterly, tiered royalty payments based on a percentage of net sales on a product-by-product basis. Corium also agreed to be responsible for and reimburse us for all of development, commercialization and regulatory expenses for any products or product candidates containing SDX, subject to certain limitations as set forth in the AZSTARYS License Agreement, including consultation fees to be paid to us for services provided to Corium in performing such activities.

In April 2021, we entered into the AZSTARYS Amendment. Pursuant to the AZSTARYS Amendment, we and Commave agreed to modify the compensation terms of the AZSTARYS License Agreement. Commave paid us $10.0 million in connection with the execution of the AZSTARYS Amendment following the FDA approval of AZSTARYS in the United States. Corium also paid us $10.0 million following the SDX scheduling determination by the DEA, which occurred on May 7, 2021. In addition, the AZSTARYS Amendment increased the total remaining future regulatory and sales milestone payments related to AZSTARYS up to an aggregate of $590.0 million. The AZSTARYS License Agreement will continue on a product-by-product basis (i) until expiration of the royalty term for the applicable product candidate in the United States and (ii) perpetually for all other countries.

In May 2021, we announced that SDX, our proprietary prodrug of d-MPH and the primary active pharmaceutical ingredient ("API") in AZSTARYS, was classified as a Schedule IV controlled substance by the DEA. AZSTARYS is classified as a Schedule II controlled substance as its formulation includes a 70:30 mixture of SDX (Schedule IV) and d-MPH (Schedule II), respectively.

During the first half of 2023, annual net sales of AZSTARYS surpassed $25 million, triggering the first annual net sales milestone payment of $5.0 million under the AZSTARYS License Agreement, which was earned and recognized as revenue in the second quarter of 2023, and received after quarter-end. During the second half of 2023, annual net sales of AZSTARYS surpassed $50 million, triggering the second milestone payment of $10.0 million under the AZSTARYS License Agreement, which was earned and recognized in the fourth quarter of 2023, and received in February 2024.

APADAZ (Withdrawn product)

The FDA approved APADAZ in February 2018. APADAZ is an immediate-release combination product containing benzhydrocodone, our prodrug of hydrocodone, and acetaminophen for the short-term (no more than 14 days) management of acute pain severe enough to require opioid analgesic and for which alternative treatments are inadequate. In October 2018, we entered into a collaboration and license agreement (the "APADAZ License Agreement") with KVK-Tech, Inc. ("KVK"), under which we granted to KVK the exclusive license to manufacture and commercialize APADAZ in the U.S. On May 31, 2023, the Company and KVK terminated the APADAZ License Agreement. Currently, the APADAZ NDA has been withdrawn and the product is not commercially available.

Our Intellectual Property

Our intellectual property ("IP") strategy includes seeking composition-of-matter patents, among other patents, for our prodrugs, product candidates and conjugates of our prodrugs while also protecting, where appropriate as trade secrets, our proprietary LAT platform technology, the process by which we identify, screen, evaluate and select ligands to be conjugated with parent drugs to create our prodrugs. Our current prodrugs all consist of an approved parent drug and one or more ligands that we have selected using our proprietary LAT platform technology. The parent drug and ligand or ligands together may potentially constitute a new molecule and thus may be eligible for composition-of-matter patent protection, among other patent protections, in the U.S. and abroad. Beyond our internally generated IP, we have also acquired extensive IP portfolios through our business development efforts which support the products and product candidates that we are seeking to commercialize and/or develop.

In addition to the execution of our IP strategy, we also depend upon the skills, knowledge and experience of our scientific and technical personnel, as well as that of our advisors, consultants and other contractors. To help protect our LAT platform technology, as well as any proprietary know-how and show-how beyond that which is patentable, we rely on trade secret protection and confidentiality agreements to protect our interests. To this end, we generally require our employees, consultants and advisors to enter into confidentiality agreements prohibiting the disclosure of confidential information and, in some cases, requiring disclosure and assignment to us of the ideas, developments, discoveries, inventions and improvements important to our business.

As of December 31, 2023, we have been granted and maintain 62 active patents within the United States, and an additional 241 active foreign patents covering our selected prodrugs and product candidates. The terms of the 62 issued U.S. patents extend to various dates ranging, for example, between 2029 and 2040. The term of our overall domestic and foreign patent portfolio related to our selected prodrugs and product candidates, including patent term adjustments but excluding possible patent term extensions, extend to various dates ranging, for example, between 2029 and 2042, if pending patent applications in each of our patent families are issued as patents. As of December 31, 2023, we had 19 pending patent applications under active prosecution in the United States, and an additional 117 pending foreign patent applications potentially covering our selected prodrugs and product candidates. Our issued and granted patents provide protection in jurisdictions that include the United States, Australia, Canada, Chile, China, European Countries, Hong Kong, India, Indonesia, Israel, Japan, Kazakhstan, Malaysia, Mexico, New Zealand, Philippines, Romania, Russia, Singapore, South Africa, South Korea, and Vietnam.

Arimoclomol

Pursuant to our acquisition of the assets of Orphazyme, we have received method of use and method of treatment patents, and have filed related patent applications, related to the arimoclomol families in various jurisdictions, including the U.S., European countries, Israel, Japan, South Korea, Canada, China, Brazil, Russia and Turkey, with anticipated patent expiration dates of 2029, excluding any potential patent term adjustments or extensions. We anticipate filing additional patent applications related to the arimoclomol families.

OLPRUVA (sodium phenylbutyrate)

We acquired the IP portfolio supporting OLPRUVA as part of the Merger with Acer. We have both U.S. and foreign patents with claims related to OLPRUVA. Our U.S. patents are directed to pharmaceutical compositions, including OLPRUVA's polymer coated, multi-particulate dosage formulation for oral administration and covers certain methods of use claims related to OLPRUVA Additionally, we have patents in Europe, Israel, and Mexico related to pharmaceutical compositions, including OLPRUVA's polymer coated, multi-particulate dosage formulation for oral administration. These patents expire in 2036.

In October 2022, the U.S. Patent and Trademark Office ("USPTO") issued a Notice of Allowance for U.S. patent application No. 16/624,834 for claims related to a kit comprising a combination therapeutic product composed of sodium phenylbutyrate or glycerol phenylbutyrate and sodium benzoate. That application has now issued as U.S. Patent No. 11,517,547 and was exclusively licensed to Acer, which we acquired in November 2023, from Baylor College of Medicine ("BCM"), with an expiration date in June 2038.

In July 2022, the China National Intellectual Property Administration ("CNIPA") issued Electronic Patent Certificate ZL202122004991.9 in May 2022, for Utility Model directed to OLPRUVA (sodium phenylbutyrate). Specifically, the patent covers dosage form claims related to OLPRUVA's polymer coated formulation for oral administration as a potential treatment for UCDs and Maple Syrup Urine Disease ("MSUD"). The patent has an expiration date in August 2031.

We have exclusive rights to certain patents and other intellectual property from BCM for the use of sodium phenylbutyrate (NaPB) for the treatment of inborn errors of BCAA metabolism, including MSUD. The licensed patents cover methods and compositions for treating humans (and animals) with various formulations and prodrugs of NaPB for inborn errors of BCAA metabolism, including MSUD, with the latest expiring in 2032. We made filings in the geographic regions that represent the largest incidence and prevalence of MSUD, including the U.S., selected countries in Europe (including Turkey), and Brazil. BCM has received three patents in the U.S. and one in the EU with respect to OLPRUVA, each of which is exclusively licensed to us pursuant to our agreement with BCM.

We also expect to benefit from potential commercial exclusivity afforded to the first drug approved after obtaining orphan drug designation for the treatment of MSUD. Orphan drug designation for OLPRUVA for the treatment of MSUD was granted by the FDA in August 2014.

Furthermore, we may qualify to receive an additional six months of pediatric exclusivity in the U.S., which runs consecutively to an existing exclusivity, if we conduct a successful pediatric study of OLPRUVA for the treatment of MSUD, approved by the FDA for this purpose.

AZSTARYS and Serdexmethylphenidate (SDX)

We have received composition-of-matter patents and also additionally filed composition-of-matter and method of treatment patent applications related to the AZSTARYS and SDX families in the United States and in Argentina, Australia, Brazil, Canada, Chile, China, Egypt, Hong Kong, European Countries, India, Israel, Indonesia, Japan, South Korea, Kazakhstan, Mexico, Malaysia, New Zealand, Philippines, Russia, Singapore, South Africa, Thailand, Ukraine, and Vietnam. We anticipate filing additional patent applications for our prodrugs and product candidates covering SDX and KP1077.

Celiprolol

The Merger with Acer included the acquisition of celiprolol, for which we intend to protect our commercial rights in the U.S. via multiple pathways. We believe celiprolol will be eligible for NCE exclusivity which provides upon approval as an NCE five years of marketing exclusivity, during which time the FDA will not approve another drug with the same active ingredient, regardless of the indication for use, in the U.S. In January 2015, the FDA granted celiprolol Orphan Drug designation, which provides seven years of marketing exclusivity for a drug intended to treat a rare condition, if approved. During the Orphan Drug exclusivity period, the FDA cannot approve the same drug for the same indication, unless it demonstrates clinical superiority. Orphan Drug exclusivity does not prevent the FDA from approving the same drug for a different indication, or a different drug for the same indication. NCE exclusivity and Orphan Drug exclusivity periods run concurrently. Furthermore, celiprolol may qualify for an additional six months of pediatric exclusivity in the U.S., which requires the submission of one or more studies in pediatric subjects that meet requirements to be specified by the FDA in a written request for pediatric studies. Pediatric exclusivity can be obtained either before or after NDA approval. Pediatric exclusivity is attached to the end of an existing exclusivity and runs consecutively. We may also consider making modifications to the formulation in order to improve the product profile and to seek additional intellectual property. While unapproved drugs may be imported into the U.S. underspecified circumstances, such as for use in clinical studies under a valid and effective investigational new drug ("IND") or for further manufacture into an IND drug or an approved drug, we intend to aggressively assert our rights, via regulatory and legal means, to limit the importation of non-FDA approved versions of celiprolol.

In 2022, the USPTO issued a Notice of Allowance for a patent application exclusively licensed from Assistance Publique—Hôpitaux de Paris (AP-HP), for claims related to certain methods of vEDS with celiprolol. This application, titled “Method of Providing Celiprolol Therapy to a Patient,” has now issued as a U.S. patent with an expiration date in November 2038.

Commercialization

In December 2022, the FDA approved OLPRUVA (sodium phenylbutyrate) for oral suspension in the U.S. OLPRUVA is a prescription medicine used along with certain therapy, including changes in diet, for the long-term management of adults and children weighing 44 pounds (20 kg) or greater and with a body surface area (“BSA”) of 1.2m^2 or greater, with UCDs, involving deficiencies of CPS, OTC or AS. OLPRUVA is not used to treat rapid increase of ammonia in the blood (acute hyperammonemia), which can be life-threatening and requires emergency medical treatment. Approximately 1 in 100,000 people have UCD, and there are an estimated 800 patients being actively treated in US. While there are therapies currently approved for the treatment of UCDs, there remains unmet needs for this patient population. Current branded products include RAVICTI®, marketed by Amgen, Inc. (previously Horizon Therapeutics) and PHEBURANE®, marketed by Medunik USA. OLPRUVA offers benefits over other sodium phenylbutrate treatments by eliminating issues with palatability, offering improved portability with its single-dose envelopes, and it comes in a dosage that personalized to the patient based on weight.

To support the launch of OLPRUVA, we have built in-house capabilities including rare disease sales specialists who are working with prescribing clinicians and healthcare providers, which include metabolic specialists and clinical geneticists, as well as marketing, patient reimbursement services, market access and contracting, patient advocacy, and medical affairs teams. We have successfully recruited, on-boarded and trained our full commercial team and full launch for OLPRUVA began January 29, 2024. We also have arrangements with third parties to provide additional services such as distribution and specialty pharmacy offerings. To support the efforts of our team members which are in the field engaging with HCPs, we are actively engaged in negotiations with the major commercial payers and state Medicaid organizations to seek access for OLPRUVA. We have established promotional programs to drive awareness and patient experience with OLPRUVA including *Quick Start,* a thirty-day free trial program designed to provide patient experience, and other patient co-pay programs, reflecting our commitment to ensure access to innovative treatments to those in need.

In March 2021, we announced that the FDA approved the NDA for AZSTARYS, a once-daily product for the treatment of ADHD in patients ranging from six years and older. Corium is leading the commercialization of AZSTARYS in the U.S. under the AZSTARYS License Agreement. Corium commercially launched AZSTARYS in the U.S. in July 2021. In December 2021, Commave Therapeutics, S.A. sublicensed to Shanghai Ark Biopharmaceutical Co., Ltd. the commercialization rights Greater China, including mainland China, Hong Kong, Macau and Taiwan.

We have established a small, targeted commercial team which is designed to fully service the patients and prescribers within the rare disease indications for which we are successful in gaining approval for our product candidates. However, if our product candidates have large potential market opportunities that would require significant marketing resources, we may conclude that the most appropriate approach to their commercialization, if they receive regulatory approval, will involve forming a commercial collaboration or strategic relationship similar to those we have entered into with Commave, or consummating some type of strategic transaction, with a larger pharmaceutical or other marketing organization. As we get closer to potential approval of our product candidates, we will work to identify and implement the most appropriate commercialization strategies that we conclude are the most desirable with regard to each specific product candidate.

Competition

Our industry is characterized by rapidly advancing technologies, intense competition and a strong emphasis on proprietary products. We will face competition and potential competition from a number of sources, including pharmaceutical and biotechnology companies, specialty pharmaceutical companies, generic drug companies, drug delivery companies and academic and research institutions. Our competitors may develop or market drugs that are more effective, more convenient, more widely used and less costly or have a better safety profile than our products or product candidates and these competitors may also have significantly more resources than us and be more successful than us in manufacturing and marketing their products. We anticipate facing intense and increasing competition as new products enter the market and advanced technologies become available.

OLPRUVA

OLPRUVA competes against several currently marketed, branded and generic forms of phenylbutyrate. In particular, RAVICTI, which is marketed by Amgen Inc. (formerly Horizon Therapeutics), and PHEBURANE®, which is marketed by Medunik USA. We are also aware that there are drug candidates in clinical development for the potential treatment of UCDs. In addition, there is the potential entrance of authorized generics for RAVICTI which could enter the market as early as July 2025.

Arimoclomol

While there are currently no approved products for the treatment of NPC in the U.S., if approved, we expect the most direct competitor with respect to arimoclomol to be ZAVESCA (miglustat), which was originally developed by Actelion Pharmaceuticals and is now owned by Johnson & Johnson. Miglustat is available as a generic product in several countries, including the U.S., where it is currently approved for the treatment of another lysosomal storage disorder, Gaucher disease. Miglustat is currently approved for the treatment of NPC in Europe, Canada, Australia, New Zealand, and several countries in Asia and South America where it is marketed as ZAVESCA and marketed as BRAZAVES in Japan. We are also aware that there are several other drug candidates in clinical development for the treatment of NPC. Furthest along of these drug candidates is IB1001 from IntraBio (UK), which recently submitted its NDA to the FDA.

Celiprolol

We are not aware of any active ongoing clinical trials for the treatment of vEDS. Aytu BioPharma, Inc. development program known as AR101/enzastaurin was indefinitely suspended in October 2022.

KP1077

If approved, we intend for KP1077 to compete against XYWAV®, marketed by Jazz Pharmaceuticals, and potentially with other products that are currently in development for the treatment of IH. KP1077 could face potential competition from any products for the treatment of IH that are currently in or which may enter into clinical development.

AZSTARYS

AZSTARYS competes against currently marketed, branded and generic methylphenidate products for the treatment of ADHD. Some of these currently marketed products include CONCERTA®, marketed by J&J Innovative Medicines (formerly Jannsen), QUELBREE®, marketed by Supernus Pharmaceuticals, Inc., QUILLIVANT XR®and QUILLICHEW ER®, marketed by Tris Pharma, RITALIN, FOCALIN® and FOCALIN XR®, marketed by Novartis AG, METADATE CD®, marketed by UCB SA, DAYTRANA®, marketed by Noven Therapeutics, LLC, Neos Therapeutics' CONTEMPLA XR-ODT®, marketed Aytu BioScience, Inc., JORNAY PM®, Ironshore Pharmaceuticals, Inc., and ADHANSIA XR®, marketed by Adlon Therapeutics, in addition to multiple other branded and generic methylphenidate products. In addition, AZSTARYS will face potential competition from any other methylphenidate products for the treatment of ADHD that are currently in, or which may enter into clinical development.

Many of our competitors either alone or with strategic partners, have or will have substantially greater financial, technical, and human resources compared with us. Accordingly, our competitors may be more successful in developing or marketing products and technologies that are more effective, safer or less costly. Additionally, our competitors may obtain regulatory approval for their products more rapidly and may achieve more widespread market acceptance. These companies also compete with us in recruiting and retaining qualified scientific and management personnel, establishing clinical study sites and patient registration for clinical studies and acquiring technologies complementary to, or necessary for, our programs. Smaller or early-stage companies may also prove to be significant competitors, particularly through collaborative arrangements with large and established companies. There are other non-pharmaceutical therapeutic approaches that are used or may be used for our targeted indications. For example, liver transplantation may be used in some cases to treat UCDs in pediatric patients who have developed acute liver failure.

Manufacturing

Our manufacturing strategy is to rely on contract manufacturers to produce our approved products and product candidates for clinical trials and, if approved, drug product for commercial sale. We currently have no manufacturing facilities and limited personnel with manufacturing experience. We have contracted with third parties for the manufacture, testing, and storage of our approved products and product candidates and intend to continue to do so in the future. We expect to contract with third-party manufacturers for the manufacture of all API supply needs outside the United States if and when we receive approval from regulatory authorities outside the United States.

Our current and any future third-party manufacturers, their facilities and all lots of drug substance and drug products used in our clinical trials are required to be in compliance with current good manufacturing practices ("cGMPs") and comparable foreign regulations. The cGMP and comparable foreign regulations include requirements relating to organization of personnel, buildings and facilities, equipment, control of components and drug product containers and closures, production and process controls, packaging and labeling controls, holding and distribution, laboratory controls, records and reports, and returned or salvaged products. The manufacturing facilities for our products must meet cGMP and comparable foreign requirements and FDA and foreign regulatory authorities' satisfaction before any product is approved and we can manufacture commercial products. Our current and any future third-party manufacturers are also subject to periodic inspections of facilities by the FDA and other authorities, including procedures and operations used in the testing and manufacture of our products to assess our compliance with applicable regulations.

Failure to comply with statutory and regulatory requirements subjects a manufacturer to possible legal or regulatory action, including refusal to approve pending applications, license suspension or revocation, withdrawal of an approval, imposition of a clinical hold or termination of clinical trials, warning letters, untitled letters, cyber letters, modification of promotional materials or labeling, product recalls, product seizures or detentions, refusal to allow imports or exports, total or partial suspension of production or distribution, debarment, injunctions, fines, consent decrees, additional reporting requirements and oversight if we become subject to a corporate integrity agreement or similar agreements to resolve allegations of non-compliance with these laws, refusals of government contracts and new orders under existing contracts, exclusion from participation in federal and state healthcare programs, restitution, disgorgement or civil or criminal penalties, including fines and individual imprisonments.

Third-Party Payor Coverage and Reimbursement

Sales of pharmaceutical products depend in significant part on the availability of coverage and adequate reimbursement by third-party payors, such as state and federal governmental authorities, including those that administer the Medicare and Medicaid programs, and private managed care organizations and health insurers. Decisions regarding the extent of coverage and amount of reimbursement to be provided for each of our product and product candidates is and will be made on a plan-by-plan basis. One payor’s determination to provide coverage for a product does not assure that other payors will also provide coverage, and adequate reimbursement, for the product. Each third-party payor determines whether or not it will provide coverage for a drug, what amount it will pay providers for the drug, and on what tier of its formulary the drug will be placed. These decisions are influenced by the existence of multiple drug products within a therapeutic class and the net cost to the plan, including the amount of the prescription price, if any, rebated by the drug’s manufacturer. Typically, generic versions of drugs are placed in a preferred tier. The position of a drug on the formulary generally determines the co-payment that a patient will need to make to obtain the drug and can strongly influence the adoption of a drug by patients and physicians. Patients who are prescribed treatments for their conditions and providers performing the prescribed services generally rely on third-party payors to reimburse all or part of the associated healthcare costs. Patients are unlikely to use our products unless coverage is provided, and reimbursement is adequate to cover a significant portion of the cost of our products. Additionally, a third-party payor’s decision to provide coverage for a drug does not imply that an adequate reimbursement rate will be approved. Also, third-party payors are developing increasingly sophisticated methods of controlling healthcare costs. As a result, coverage, reimbursement and placement determinations are complex and are often the subject of extensive negotiations between the payer and the owner of the drug.

Unless we enter into a strategic collaboration under which our collaborator assumes responsibility for seeking coverage and reimbursement for a given product (such as the AZSTARYS License Agreement), we will be responsible for negotiating coverage, reimbursement and placement decisions for our product candidates, if approved. Coverage, reimbursement and placement decisions for a new product are based on many factors including the coverage, reimbursement and placement of already marketed branded drugs for the same or similar indications, the safety and efficacy of the new product, availability of generics for similar indications, the clinical need for the new product and the cost-effectiveness of the product. Increasingly, both purchasers and payors are also conducting comparative clinical and cost effectiveness analyses involving application of metrics, including data on patient outcomes, provided by manufacturers.

Within the Medicare program, as self-administered drugs, our product and product candidates would be reimbursed under the expanded prescription drug benefit known as Medicare Part D. This program is a voluntary Medicare benefit administered by private plans that operate under contracts with the federal government. These plans develop formularies that determine which products are covered and what co-pay will apply to covered drugs. The plans have considerable discretion in establishing formularies and tiered co-pay structures, negotiating rebates with manufacturers and placing prior authorization and other restrictions on the utilization of specific products, subject to review by the Centers for Medicare & Medicaid Services ("CMS"), for discriminatory practices. These Part D plans negotiate discounts with drug manufacturers, which are passed on, in whole or in part, to each of the plan's enrollees through reduced premiums. Historically, Part D beneficiaries have been exposed to significant out-of-pocket costs after they surpass an annual coverage limit and until they reach a catastrophic coverage threshold. However, changes made by recent legislation will reduce this patient coverage gap, known as the "donut hole", by transitioning patient responsibility in that coverage range from 100% in 2010 to only 25% currently. To help achieve this reduction, pharmaceutical manufacturers are required to provide quarterly discounts of 70%, which commenced January 1, 2019. In 2020, drug manufacturers became responsible for a larger share of total drug costs due to an increase to the catastrophic threshold. Such increase also resulted in a higher out-of-pocket threshold paid by Part D beneficiaries.

If a drug product is available for reimbursement by Medicare or Medicaid, its manufacturer must comply with various health regulatory requirements and price reporting metrics, which may include, as applicable, the Medicare Prescription Drug, Improvement, and Modernization Act of 2003, the Medicaid rebate requirements of the Omnibus Budget Reconciliation Act of 1990 (the "OBRA"), and the Veterans Health Care Act of 1992 (the "VHCA"), each as amended. Among other things, the OBRA requires drug manufacturers with certain drugs covered by Medicaid to pay rebates on prescription drugs to state Medicaid programs. States may also negotiate "supplemental" Medicaid rebates on drug products dispensed under Medicaid. Manufacturers participating in Medicaid are also generally required to participate in the Public Health Service 340B Drug Discount Program, which imposes a mandatory discount on purchases by certain customers. Manufacturers of innovator drugs, including 505(b)(2) drugs, that participate in the Medicaid program are also required to offer the drugs on the Federal Supply Schedule purchasing program of the General Services Administration for purchase by the Department of Veterans Affairs, the Department of Defense and other authorized users at a mandatory discount. Additional laws and requirements apply to these contracts. Participation in such federal programs may result in prices for our future products that will likely be lower than the prices we might otherwise obtain.

Third-party payers, including the U.S. government, continue to apply downward pressure on the reimbursement of pharmaceutical products. Also, the trend towards managed health care in the United States and the concurrent growth of organizations such as health maintenance organizations may result in lower reimbursement for pharmaceutical products. We expect that these trends will continue as these payors implement various proposals or regulatory policies, including various provisions of the recent health reform legislation that affect reimbursement of these products. There are currently, and we expect that there will continue to be, a number of federal, state and foreign proposals to implement controls on reimbursement and pricing, directly and indirectly.

In international markets, reimbursement and healthcare payment systems vary significantly by country, and many countries have instituted price ceilings on specific products and therapies. For example, in the E.U., pricing and reimbursement schemes vary widely from country to country. Some countries may require the completion of additional studies that compare the cost-effectiveness of a particular medicinal product candidate to currently available therapies. This Health Technology Assessment ("HTA") which is currently governed by the national laws of the individual EU member states, is the procedure according to which the assessment of the public health impact, therapeutic impact and the economic and societal impact of use of a given medicinal product in the national healthcare systems of the individual country is conducted. The outcome of HTA regarding specific medicinal products will often influence the pricing and reimbursement status granted to these medicinal products by the competent authorities of individual EU member states. The downward pressure on healthcare costs in general, particularly prescription medicines, has become very intense. Pharmaceutical products may face competition from lower-priced products in foreign countries that have placed price controls on pharmaceutical products and may also compete with imported foreign products. Furthermore, there is no assurance that a product will be considered medically reasonable and necessary for a specific indication, will be considered cost-effective by third-party payors, that an adequate level of reimbursement will be established even if coverage is available or that the third-party payors' reimbursement policies will not adversely affect the ability for manufacturers to sell products profitably. Historically, products launched in the E.U. do not follow the price structures which prevail in the U.S., and generally, prices tend to be significantly lower.

Government Regulation

The FDA and comparable regulatory agencies in state and local jurisdictions and in foreign countries impose substantial requirements upon the clinical development, manufacture and marketing of pharmaceutical products. These agencies and other federal, state and local entities regulate research and development activities and the testing, manufacture, quality control, safety, effectiveness, labeling, storage, packaging, recordkeeping, tracking, approval, import, export, distribution, advertising and promotion of our products and product candidates. A new drug must be approved by the FDA through the NDA process before it may be legally marketed in the United States. The process of obtaining regulatory approvals and the subsequent compliance with applicable federal, state, local and foreign statutes and regulations require the expenditure of substantial time and financial resources.

The process required by the FDA before product candidates may be marketed in the United States generally involves the following:

- non-clinical laboratory and animal tests that must be conducted in accordance with good laboratory practices ("GLP") requirements and other applicable regulations;
- submission of an IND, which must be received by the FDA and become effective before human clinical trials may begin;
- approval by an independent institutional review board ("IRB") or ethics committee at each clinical site or centrally before each trial may be initiated;
- performance of adequate and well-controlled human clinical trials to establish the safety and efficacy of the proposed product candidate for its intended use, performed in accordance with good clinical practice requirements ("GCPs");
- preparation and submission of a NDA to the FDA;
- satisfactory completion of an FDA advisory committee review, if applicable;
- pre-approval inspection of manufacturing facilities at which the drug is produced to assess their compliance with cGMPs and of selected clinical investigation sites to assess compliance with GCPs; and
- FDA approval of an NDA to permit commercial marketing for particular indications for use.

Clinical Trials

The testing and regulatory approval process requires substantial time, effort and financial resources. Preclinical studies include laboratory evaluation of drug substance chemistry, pharmacology, toxicity and drug product formulation, as well as animal studies to assess potential safety and efficacy. Prior to commencing the first human clinical trial with a product candidate, we must submit the results of the preclinical tests and preclinical literature, together with manufacturing information, analytical data and any available clinical data or literature, among other things, to the FDA as part of an IND. An IND is a request for authorization from the FDA to administer an investigational new drug product to humans. The central focus of an IND submission is on the general investigational plan and the protocol(s) for clinical studies.

The IND becomes effective 30 days after receipt by the FDA, unless the FDA, within the 30-day time period, raises safety concerns or questions about the conduct of the clinical trial by imposing a clinical hold. In such a case, the IND sponsor and the FDA must resolve any outstanding concerns before the clinical trial can begin. Submission of an IND may not result in FDA authorization to commence a clinical trial.

Clinical trials involve the administration of the investigational product to human subjects under the supervision of qualified investigators in accordance with GCPs, which include the requirement that all research subjects provide their informed consent for their participation in any clinical study. Clinical trials are conducted under protocols detailing, among other things, the objectives of the study, the parameters to be used in monitoring safety and the effectiveness criteria to be evaluated. A separate submission to the existing IND must be made for each successive clinical trial conducted during product development, as well as amendments to previously submitted clinical trials. Further, an independent IRB for each study site proposing to conduct the clinical trial must review and approve the plan for any clinical trial, its informed consent form and other communications to study subjects before the clinical trial commences at that site. The IRB must continue to oversee the clinical trial while it is being conducted, including any changes to the study plans. Regulatory authorities, an IRB or the sponsor may suspend or discontinue a clinical trial at any time on various grounds, including a finding that the subjects are being exposed to an unacceptable health risk, the clinical trial is not being conducted in accordance with the FDA's or the IRB's requirements, if the drug has been associated with unexpected serious harm to subjects, or based on evolving business objectives or competitive climate. Some studies also include a data safety monitoring board, which receives special access to unblinded data during the clinical trial and may advise us to halt the clinical trial if it determines that there is an unacceptable safety risk for subjects or other grounds, such as no demonstration of efficacy. Depending on its charter, this group may determine whether a trial may move forward at designated check points based on access to certain data from the trial. The FDA or the sponsor may suspend a clinical trial at any time on various grounds, including a finding that the research subjects or patients are being exposed to an unacceptable health risk. Similarly, an IRB can suspend or terminate approval of a clinical trial at its institution if the clinical trial is not being conducted in accordance with the IRB's requirements or if the drug has been associated with unexpected serious harm to patients. There are also requirements governing the reporting of ongoing clinical studies and clinical study results to public registries.

In general, for purposes of NDA approval, human clinical trials are typically conducted in three sequential phases that may overlap.

- *Phase 1* - The product candidate is initially introduced into healthy subjects or patients with the target disease or condition. These studies are conducted to test the product candidate for safety, dosage tolerance, structure-activity relationships, mechanism of action, absorption, metabolism, distribution and excretion. If possible, Phase 1 trials may also be used to gain early evidence of product effectiveness.
- *Phase 2* - The product candidate is administered to a limited patient population with a specified disease or condition to evaluate the preliminary efficacy, optimal dosage and dosing schedule and to identify possible adverse side effects and safety risks. Multiple Phase 2 clinical trials may be conducted to obtain information prior to beginning larger and more expensive Phase 3 clinical trials.
- *Phase 3* - The product candidate is administered to an expanded patient population to further evaluate dosage to provide statistically significant evidence of clinical efficacy and to further test for safety in an expanded subject population at multiple clinical trial sites. These clinical trials are intended to establish the overall risk/benefit ratio of the product and provide an adequate basis for product labeling.

In the case of a 505(b)(2) NDA, some of the above-described studies and preclinical studies may not be required or may be abbreviated. Pharmacokinetic and other bridging studies may be needed, however, to demonstrate the relevance of the studies that were previously conducted by other sponsors to the drug that is the subject of the NDA.

The FDA may require, or companies may pursue, additional clinical trials after a product is approved. These so-called Phase 4, or post-market, studies may be used to gain additional experience from the treatment of patients in the intended therapeutic indication. In certain instances, the FDA may mandate the performance of Phase 4 clinical trials as a condition of approval of an NDA.

The manufacture of investigational drugs for the conduct of human clinical trials is subject to cGMP requirements. Investigational drugs and active pharmaceutical ingredients imported into the United States are also subject to regulation by the FDA relating to their labeling and distribution. Further, the export of investigational drug products outside of the United States is subject to regulatory requirements of the receiving country as well as U.S. export requirements under the FFDCA. In addition, while the IND is active and before approval, progress reports summarizing the results of the clinical trials and nonclinical studies performed since the last progress report must be submitted at least annually to the FDA, and written IND safety reports must be submitted to the FDA and investigators for serious and unexpected suspected adverse events, findings from other studies suggesting a significant risk to humans exposed to the same or similar drugs, findings from animal or in vitro testing suggesting a significant risk to humans, and any clinically important increased incidence of a serious suspected adverse reaction compared to that listed in the protocol or investigator's brochure.

Concurrent with clinical trials, companies usually complete additional animal studies and must also develop additional information about the chemistry and physical characteristics of the product candidate as well as finalize a process for manufacturing the product in commercial quantities in accordance with cGMP requirements. The manufacturing process must be capable of consistently producing quality batches of the product candidate and, among other things, must develop methods for testing the identity, strength, quality and purity of the final product. Additionally, appropriate packaging must be selected and tested, and stability studies must be conducted to demonstrate that the product candidate does not undergo unacceptable deterioration over its shelf life.

NDA Submission and Review by the FDA

Assuming successful completion of the required clinical and preclinical testing, among other items, the results of product development, including chemistry, manufacture and controls, non-clinical studies and clinical trials are submitted to the FDA, along with proposed labeling, as part of an NDA requesting approval to market the product. Data can come from company-sponsored clinical studies intended to test the safety and effectiveness of a use of the product, or from a number of alternative sources, including studies initiated by independent investigators. The submission of an NDA requires payment of a substantial application user fee to the FDA. These user fees must be filed at the time of the first submission of the application, even if the application is being submitted on a rolling basis. Fee waivers or reductions are available in some circumstances.

In addition, under the Pediatric Research Equity Act ("PREA"), an NDA or supplement to an NDA for a new active ingredient, indication, dosage form, dosage regimen or route of administration must contain data that are adequate to assess the safety and efficacy of the drug for the claimed indications in all relevant pediatric subpopulations, and to support dosing and administration for each pediatric subpopulation for which the product is safe and effective. The FDA may, on its own initiative or at the request of the applicant, grant deferrals for submission of some or all pediatric data until after approval of the product for use in adults or full or partial waivers from the pediatric data requirements.

The FDA may refer drugs which present difficult questions of safety or efficacy to an advisory committee. An advisory committee is a panel that typically includes clinicians and other experts who review, evaluate and make a recommendation as to whether the application should be approved and under what conditions. The FDA is not bound by the recommendations of an advisory committee, but it considers such recommendations carefully when making decisions.

Once the FDA receives an application, it has 60 days to review the NDA to determine if it is substantially complete to permit a substantive review, before it accepts the application for filing. Once the submission is accepted for filing, the FDA begins an in-depth review of the NDA. The timeline for the FDA to complete its review of a NDA may differ based on whether the application is a standard review or priority review application. The FDA may give a priority review to applications for drugs that are intended to treat serious conditions and if approved, would provide significant improvements in the safety or effectiveness of the treatment, diagnosis, or prevention of such serious conditions. Under the goals and policies agreed to by the FDA under the Prescription Drug User Fee Act ("PDUFA"), the FDA has set the review goal of ten months from the 60-day filing date to complete its initial review of a standard NDA for a new molecular entity, or NME, and make a decision on the application. For non-NME standard applications, the FDA has set the review goal of ten months from the submission date to complete its initial review and to make a decision on the application. For priority review applications, the FDA has set the review goal of reviewing NME NDAs within six months of the 60-day filing date and non-NME applications within six months of the date of receipt. Such deadlines are referred to as the PDUFA date. The review process and the PDUFA date may also be extended if the FDA requests or the NDA sponsor otherwise provides additional information or clarification regarding the submission.

The FDA reviews applications to determine, among other things, whether a product is safe and effective for its intended use and whether the manufacturing controls are adequate to assure and preserve the product's identity, strength, quality and purity. Before approving an NDA, the FDA will inspect the facility or facilities where the product is manufactured. The FDA will not approve an application unless it determines that the manufacturing processes and facilities, including contract manufacturers and subcontracts, are in compliance with cGMP requirements and adequate to assure consistent production of the product within required specifications. Additionally, before approving an NDA, the FDA will typically inspect one or more clinical trial sites to assure compliance with GCPs.

Once the FDA's review of the application is complete, the FDA will issue either a CRL or approval letter. A CRL indicates that the review cycle of the application is complete, and the application cannot be approved in its current form. A CRL will describe all of the deficiencies that the FDA has identified in the NDA, except that where the FDA determines that the data supporting the application are inadequate to support approval, the FDA may issue the CRL without first conducting required inspections and/or reviewing proposed labeling. In issuing the CRL, the FDA may recommend actions that the applicant might take to place the NDA in condition for approval, including requests for additional information or clarification. Even with the submission of additional information, the FDA ultimately may decide that the application does not satisfy the regulatory criteria for approval. If and when those conditions have been met to the FDA's satisfaction, the FDA may issue an approval letter. An approval letter authorizes commercial marketing of the drug with specific prescribing information for specific indications.

The FDA may delay or refuse approval of an NDA if applicable regulatory criteria are not satisfied, require additional testing or information and/or require post-marketing testing and surveillance to monitor safety or efficacy of a product, or impose other conditions, including distribution restrictions or other risk management mechanisms. For example, the FDA may require a risk evaluation and mitigation strategy ("REMS"), as a condition of approval or following approval. A REMS is a safety strategy to manage a known or potential serious risk associated with a medicine and to enable patients to have continued access to such medicines by managing their safe use, and could include medication guides, physician communication plans, or elements to assure safe use, such as restricted distribution methods, patient registries, and other risk minimization tools.

If regulatory approval of a product is granted, such approval may entail limitations on the indicated uses for which such product may be marketed or may include contraindications, warnings or precautions in the product labeling, including a boxed warning. If the FDA requires a boxed warning, the sponsor may also be subject to specified promotional restrictions, such as the prohibition of reminder advertisements. The FDA also may not approve the inclusion of labeling claims necessary for successful marketing. Once approved, the FDA may withdraw the product approval if compliance with pre- and post-marketing regulatory standards is not maintained or if problems occur after the product reaches the marketplace. In addition, the FDA may require Phase 4 post-marketing studies to monitor the effect of approved products and may limit further marketing of the product based on the results of these post-marketing studies.

Advertising and Promotion

The FDA and other federal regulatory agencies closely regulate the marketing and promotion of drugs through, among other things, guidance and regulations for direct-to-consumer advertising, communications regarding unapproved uses, industry-sponsored scientific and educational activities, and promotional activities involving the internet. A product cannot be commercially promoted before it is approved. After approval, product promotion can include only those claims relating to safety and efficacy that are consistent with the labeling approved by the FDA. Healthcare providers are permitted to prescribe drugs for “off-label” uses — that is, uses not approved by the FDA and therefore not described in the drug’s labeling because the FDA does not regulate the practice of medicine. However, FDA regulations impose stringent restrictions on manufacturers’ communications regarding off-label uses. Failure to comply with applicable FDA requirements and restrictions in this area may subject a company to adverse publicity and enforcement action by the FDA, the U.S. Department of Justice, or the Office of the Inspector General of HHS, as well as state authorities. This could subject a company to a range of penalties that could have a significant commercial impact, including civil and criminal fines and agreements that materially restrict the manner in which a company promotes or distributes drug products.

Post-Approval Requirements

Any products manufactured or distributed pursuant to FDA approvals are subject to continuing regulation by the FDA, including manufacturing, periodic reporting, product sampling and distribution, advertising, promotion, drug shortage reporting, compliance with any post-approval requirements imposed as a conditional of approval such as Phase 4 clinical trials, REMS and surveillance, recordkeeping and reporting requirements, including adverse experiences.

After approval, most changes to the approved product, such as adding new indications or other labeling claims are subject to prior FDA review and approval. There also are continuing, annual human prescription drug program fee requirements for approved products. Drug manufacturers and their subcontractors are required to register their establishments with the FDA and certain state agencies and to list their drug products and are subject to periodic announced and unannounced inspections by the FDA and these state agencies for compliance with cGMPs and other requirements, which impose procedural and documentation requirements upon us and our third-party manufacturers. We cannot be certain that we or our present or future suppliers will be able to comply with the cGMP regulations and other FDA regulatory requirements.

Changes to the manufacturing process are strictly regulated and often require prior FDA approval before being implemented, or FDA notification. FDA regulations also require investigation and correction of any deviations from cGMPs and specifications and impose reporting and documentation requirements upon the sponsor and any third-party manufacturers that the sponsor may decide to use. Accordingly, manufacturers must continue to expend time, money and effort in the area of production and quality control to maintain cGMP compliance.

Later discovery of previously unknown problems with a product, including adverse events of unanticipated severity or frequency, or with manufacturing processes, or failure to comply with regulatory requirements, may result in withdrawal of marketing approval, mandatory revisions to the approved labeling to add new safety information or other limitations, imposition of post-market studies or clinical trials to assess new safety risks, or imposition of distribution or other restrictions under a REMS program, among other consequences.

The FDA closely regulates the marketing and promotion of drugs. A company can make only those claims relating to safety and efficacy, purity and potency that are approved by the FDA. Physicians, in their independent professional medical judgment, may prescribe legally available products for uses that are not described in the product’s labeling and that differ from those tested by us and approved by the FDA. Physicians may believe that such off-label uses are the best treatment for many patients in varied circumstances. The FDA does not regulate the behavior of physicians in their choice of treatments. The FDA does, however, restrict manufacturer’s communications on the subject of off-label use of their products. However, companies may share truthful and not misleading information that is otherwise consistent with a product’s FDA-approved labelling.

In addition, the distribution of prescription pharmaceutical products, including samples, is subject to the Prescription Drug Marketing Act ("PDMA"), which regulates the distribution of drugs and drug samples at the federal level, and sets minimum standards for the registration and regulation of drug distributors by the states. Both the PDMA and state laws limit the distribution of prescription pharmaceutical product samples and impose requirements to ensure accountability in distribution. The Drug Supply Chain Security Act also imposes obligations on manufacturers of pharmaceutical products related to product tracking and tracing.

Failure to comply with any of the FDA’s requirements could result in significant adverse enforcement actions. These include a variety of administrative or judicial sanctions, such as refusal to approve pending applications, withdrawal of an approval, imposition of a clinical hold or termination of clinical trials, warning letters, untitled letters, modification of promotional materials or labeling, product recalls, product seizures or detentions, refusal to allow imports or exports, total or partial suspension of production or distribution, debarment, injunctions, fines, consent decrees, additional reporting requirements and oversight if we become subject to a corporate integrity agreement or similar agreement to resolve allegations of non-compliance with these laws, refusals of government contracts and new orders under existing contracts, exclusion from participation in federal and state healthcare programs, restitution, disgorgement or civil or criminal penalties, including fines and individual imprisonment. Any of these sanctions could result in adverse publicity, among other adverse consequences.

Orphan Drug Designation and Exclusivity

Under the Orphan Drug Act, the FDA may grant orphan designation to a drug intended to treat a rare disease or condition, defined as a disease or condition with a patient population of fewer than 200,000 individuals in the U.S., or a patient population greater than 200,000 individuals in the U.S. and when there is no reasonable expectation that the cost of developing and making available the drug in the U.S. will be recovered from sales in the United States for that drug. Orphan drug designation must be requested before submitting an NDA. After the FDA grants orphan drug designation, the generic identity of the therapeutic agent and its potential orphan use are disclosed publicly by the FDA.

If a product that has orphan drug designation subsequently receives the first FDA approval for a particular active ingredient for the disease for which it has such designation, the product is entitled to orphan product exclusivity, which means that the FDA may not approve any other applications, including a full NDA, to market the same drug for the same indication for seven years, except in limited circumstances, such as a showing of clinical superiority to the product with orphan drug exclusivity or if the FDA finds that the holder of the orphan drug exclusivity has not shown that it can assure the availability of sufficient quantities of the orphan drug to meet the needs of patients with the disease or condition for which the drug was designated. Orphan drug exclusivity does not prevent the FDA from approving a different drug for the same disease or condition, or the same drug for a different disease or condition. Among the other benefits of orphan drug designation are tax credits for certain research and a waiver of the NDA application user fee.

A designated orphan drug may not receive orphan drug exclusivity if it is approved for a use that is broader than the indication for which it received orphan designation. In addition, orphan drug exclusive marketing rights in the United States may be lost if the FDA later determines that the request for designation was materially defective or, as noted above, if a second applicant demonstrates that its product is clinically superior to the approved product with orphan exclusivity or the manufacturer of the approved product is unable to assure sufficient quantities of the product to meet the needs of patients with the rare disease or condition.

Hatch-Waxman Act

Section 505 of the FFDCA describes three types of marketing applications that may be submitted to the FDA to request marketing authorization for a new drug. A Section 505(b)(1) NDA is an application that contains full reports of investigations of safety and efficacy. A 505(b)(2) NDA is an application that contains full reports of investigations of safety and efficacy but where at least some of the information required for approval comes from investigations that were not conducted by or for the applicant and for which the applicant has not obtained a right of reference or use from the person by or for whom the investigations were conducted. This regulatory pathway enables the applicant to rely, in part, on the FDA's prior findings of safety and efficacy for an existing product, or published literature, in support of its application. The FDA may also require 505(b)(2) applicants to perform additional studies or measurements to support any changes from the reference listed drug. The FDA may then approve the new product candidate for all or some of the labeled indications for which the referenced product has been approved, as well as for any new indication sought by the 505(b)(2) applicant. Section 505(j) establishes an abbreviated approval process for a generic version of approved drug products through the submission of an abbreviated new drug application ("ANDA"). An ANDA provides for marketing of a generic drug product that has the same active ingredients, dosage form, strength, route of administration, labeling, performance characteristics and intended use, among other things, to a previously approved product. ANDAs are termed "abbreviated" because they are generally not required to include preclinical and clinical data to establish safety and efficacy. Instead, generic applicants must scientifically demonstrate that their product is bioequivalent to, or performs in the same manner as, the innovator drug through in vitro, in vivo, or other testing. The generic version must deliver the same amount of active ingredients into a subject's bloodstream in the same amount of time as the innovator drug and can often be substituted by pharmacists under prescriptions written for the reference listed drug.

In seeking approval for a drug through an NDA, including a 505(b)(2) NDA, applicants are required to list with the FDA patents whose claims cover the applicant's product. Upon approval of an NDA, each of the patents listed in the application for the drug is then published in the Orange Book. These products may be cited by potential competitors in support of approval of an ANDA or 505(b)(2) NDA.

Any applicant who files an ANDA seeking approval of a generic equivalent version of a drug listed in the Orange Book or a 505(b)(2) NDA referencing a drug listed in the Orange Book must certify to the FDA that (1) no patent information on the drug or method of use that is the subject of the application has been submitted to the FDA; (2) such patent has expired; (3) the date on which such patent expires; or (4) such patent is invalid or will not be infringed upon by the manufacture, use or sale of the drug product for which the application is submitted. This last certification is known as a Paragraph IV certification. Generally, the ANDA or 505(b)(2) NDA cannot be approved until all listed patents have expired, except where the ANDA or 505(b)(2) NDA applicant challenges a listed patent through a Paragraph IV certification. If the applicant does not challenge the listed patents or does not indicate that it is not seeking approval of a patented method of use, the ANDA or 505(b)(2) NDA application will not be approved until all of the listed patents claiming the referenced product have expired, or, if permissible, are carved out.

If the ANDA or 505(b)(2) NDA applicant has provided a Paragraph IV certification to the FDA, the applicant must also send notice of the Paragraph IV certification to the holder of the NDA for the reference listed drug and the patent owner once the application has been accepted for filing by the FDA. The NDA holder or patent owner may then initiate a patent infringement lawsuit in response to the notice of the Paragraph IV certification. The filing of a patent infringement lawsuit within 45 days of the receipt of a Paragraph IV certification prevents the FDA from approving the application until the earlier of 30 months from the date of the lawsuit, expiration of the patent, settlement of the lawsuit, a decision in the infringement case that is favorable to the applicant or such shorter or longer period as may be ordered by a court. This prohibition is generally referred to as the 30-month stay. In instances where an ANDA or 505(b)(2) NDA applicant files a Paragraph IV certification, the NDA holder or patent owner regularly take action to trigger the 30-month stay, recognizing that the related patent litigation may take many months or years to resolve. Thus, approval of an ANDA or 505(b)(2) NDA could be delayed for a significant period of time depending on the patent certification the applicant makes and the reference drug sponsor's decision to initiate patent litigation. The applicant may also elect to submit a statement certifying that its proposed label does not contain, or carves out, any language regarding the patented method-of-use rather than certify to a listed method-of-use patent.

The FDA provides periods of regulatory exclusivity, which provides the holder of an approved NDA limited protection from new competition in the marketplace for the innovation represented by its approved drug for a period of three or five years following the FDA's approval of the NDA. Five years of exclusivity are available to new chemical entities ("NCEs"). An NCE is a drug that contains no active moiety that has been approved by the FDA in any other NDA. An active moiety is the molecule or ion, excluding those appended portions of the molecule that cause the drug to be an ester, salt, including a salt with hydrogen or coordination bonds, or other noncovalent derivatives, such as a complex, chelate, or clathrate, of the molecule, responsible for the therapeutic activity of the drug substance. During the exclusivity period, the FDA may not accept for review or approve an ANDA or a 505(b)(2) NDA submitted by another company that contains the previously approved active moiety. An ANDA or 505(b)(2) application, however, may be submitted one year before NCE exclusivity expires if a Paragraph IV certification is filed. Applicants may also seek to carve out certain drug labeling that is protected by exclusivity.

If a product is not eligible for the NCE exclusivity, it may be eligible for three years of exclusivity. Three-year exclusivity is available to the holder of an NDA, including a 505(b)(2) NDA, for a particular condition of approval, or change to a marketed product, such as a new formulation for a previously approved product, if one or more new clinical trials, other than bioavailability or bioequivalence trials, was essential to the approval of the application and was conducted or sponsored by the applicant. This three-year exclusivity period protects against FDA approval of ANDAs and 505(b)(2) NDAs for the condition of the new drug's approval. Five-year and three-year exclusivity will not delay the submission or approval of a full NDA; however, an applicant submitting a full NDA would be required to conduct or obtain a right of reference to all of the preclinical studies and adequate and well-controlled clinical trials necessary to demonstrate safety and efficacy.

DEA Regulation

Our products and certain of our product candidates are, or if approved, will be regulated as "controlled substances" as defined in the Controlled Substances Act of 1970 ("CSA"), and the DEA's implementing regulations, which establish registration, security, recordkeeping, reporting, storage, distribution, importation, exportation, inventory, quota and other requirements administered by the DEA. These requirements are directly applicable to us and also applicable to our contract manufacturers and to distributors, prescribers and dispensers of our product candidates. The DEA regulates the handling of controlled substances through a closed chain of distribution. This control extends to the equipment and raw materials used in their manufacture and packaging in order to prevent loss and diversion into illicit channels of commerce.

The DEA regulates controlled substances as Schedule I, II, III, IV or V substances. Schedule I substances by definition have no established medicinal use and may not be marketed or sold in the United States. A pharmaceutical product may be listed as Schedule II, III, IV or V, with Schedule II substances considered to present the highest risk of abuse and Schedule V substances the lowest relative risk of abuse among such substances. Schedule II drugs are those that meet the following criteria:

- the drug has a high potential for abuse;
- the drug has a currently accepted medical use in treatment in the United States or a currently accepted medical use with severe restrictions; and
- abuse of the drug may lead to severe psychological or physical dependence.

AZSTARYS is listed as Schedule II controlled substances under the CSA. For Schedule II controlled substances, the importation of APIs for our product candidates, as well as the manufacture, shipping, storage, sales and use of the products, are subject to a high degree of regulation. In addition to maintaining an importer and/or exporter registration, importers and exporters of controlled substances must obtain a permit for every import of a Schedule I or II substance and a narcotic substance in Schedule III, IV and V, as well as every export of a Schedule I or II substance and a narcotic substance in Schedule III and IV. For all other drugs in Schedule III, IV and V, importers and exporters must submit an import or export declaration. Schedule II drugs are subject to the strictest requirements for registration, security, recordkeeping and reporting. Also, distribution and dispensing of these drugs are highly regulated. For example, all Schedule II drug prescriptions must be signed by a physician, physically presented to a pharmacist and may not be refilled without a new prescription. Electronic prescriptions may also be permissible depending on the state, so long as the prescription complies with the DEA's requirements for electronic prescriptions.

SDX, which is the sole API in KP1077, has been listed as a Schedule IV controlled substance under the CSA. Controlled substances classified in Schedule III, IV, and V are also subject to registration, recordkeeping, reporting, and security requirements. For example, Schedule III drug prescriptions must be authorized by a physician and may not be refilled more than six months after the date of the original prescription or more than five times. A prescription for controlled substances classified in Schedules III, IV, and V issued by a physician, may be communicated either orally, in writing or by facsimile to the pharmacies. Controlled substances that are also classified as narcotics, such as hydrocodone, oxycodone and hydromorphone, are also subject to additional DEA requirements, such as manufacturer reporting of the import of narcotic raw material.

Annual registration is required for any facility that manufactures, distributes, dispenses, imports or exports any controlled substance. The registration is specific to the particular location, activity and controlled substance schedule. For example, separate registrations are needed for import and manufacturing, and each registration will specify which schedules of controlled substances are authorized. Similarly, separate registrations are also required for separate facilities. Acquisition and distribution transactions must also be reported for Schedule I and II controlled substances, as well as Schedule III narcotic substances.

The DEA typically inspects a facility to review its security measures prior to issuing a registration and on a periodic basis. Security requirements vary by controlled substance schedule, with the most stringent requirements applying to Schedule I and Schedule II substances. Required security measures include background checks on employees and physical control of inventory through measures such as cages, surveillance cameras and inventory reconciliations. Records must be maintained for the handling of all controlled substances, and periodic reports made to the DEA, for example distribution reports for Schedule I and II controlled substances, Schedule III substances that are narcotics, and other designated substances. Reports must also be made for thefts or losses of any controlled substance, and to obtain authorization to destroy any controlled substance. In addition, special permits and notification requirements apply to imports and exports of narcotic drugs. To enforce these requirements, the DEA conducts periodic inspections of registered establishments that handle controlled substances. Failure to maintain compliance with applicable requirements, particularly as manifested in loss or diversion, can result in administrative, civil or criminal enforcement action that could have a material adverse effect on our business, results of operations and financial condition. The DEA may seek civil penalties, refuse to renew necessary registrations, or initiate administrative proceedings to revoke those registrations. In some circumstances, violations could result in criminal proceedings.

In addition, a DEA quota system controls and limits the availability and production of controlled substances in Schedule I or II. Distributions of any Schedule I or II controlled substance or Schedule III narcotic must also be accompanied by special order forms, with copies provided to the DEA. Because AZSTARYS and our product candidates may be regulated as Schedule II controlled substances, they may be subject to the DEA's production and procurement quota scheme. The DEA establishes annually an aggregate quota for how much of a controlled substance may be produced in total in the United States based on the DEA's estimate of the quantity needed to meet legitimate scientific and medicinal needs. The limited aggregate amount of stimulants that the DEA allows to be produced in the U.S. each year is allocated among individual companies, which must submit applications annually to the DEA for individual production and procurement quotas. We and our contract manufacturers must receive an annual quota from the DEA in order to produce or procure any Schedule I or Schedule II substances for use in manufacturing of our product candidates. The DEA may adjust aggregate production quotas and individual production and procurement quotas from time to time during the year, although the DEA has substantial discretion in whether or not to make such adjustments. Our, or our contract manufacturers', quota of an active ingredient may not be sufficient to meet commercial demand or complete clinical trials. Any delay, limitation or refusal by the DEA in establishing our, or our contract manufacturers', quota for controlled substances could delay or stop our clinical trials or product launches, which could have a material adverse effect on our business, financial position and results of operations.

Individual states also independently regulate controlled substances. We and our contract manufacturers will be subject to state regulation on distribution of these products, including, for example, state requirements for licensures or registration.

Other Healthcare Regulatory Frameworks

Our business activities, including but not limited to, research, sales, promotion, distribution, medical education and other activities are subject to regulation by numerous regulatory and law enforcement authorities in the United States in addition to the FDA, including potentially the Department of Justice, the U.S. Department of Health and Human Services and its various divisions, including the CMS and the Health Resources and Services Administration, the Department of Veterans Affairs, the Department of Defense and state and local governments. Our business activities must comply with numerous healthcare laws, including those described below. Compliance with government regulations requires the expenditure of substantial time and financial resources.

The federal Anti-Kickback Statute prohibits, among other things, any person or entity, from knowingly and willfully offering, paying, soliciting or receiving any remuneration, directly or indirectly, overtly or covertly, in cash or in kind, to induce or reward, or in return for, the referral of an individual for, or purchasing, leasing, ordering, or arranging for the purchase, lease or order of, any good, facility, item or service reimbursable under Medicare, Medicaid or other federal healthcare programs. The term remuneration has been interpreted broadly to include anything of value. There are a number of statutory exceptions and regulatory safe harbors protecting some common activities from prosecution. The exceptions and safe harbors are drawn narrowly and practices that involve remuneration that may be alleged to be intended to induce prescribing, purchasing or recommending may be subject to scrutiny if they do not qualify for an exception or safe harbor. Failure to meet all of the requirements of a particular applicable statutory exception or regulatory safe harbor does not make the conduct per se illegal under the federal Anti-Kickback Statute. Instead, the legality of the arrangement will be evaluated on a case-by-case basis based on a cumulative review of all of its facts and circumstances. Additionally, a person or entity does not need to have actual knowledge of the federal Anti-Kickback Statute, or the specific intent to violate it to have committed a violation.

The federal civil and criminal false claims laws, including the federal False Claims Act, which can be enforced by private citizens through civil whistleblower or *qui tam* actions, prohibit, among other things, any person or entity from knowingly presenting, or causing to be presented, a false claim for payment to, or approval by, the federal government, including the Medicare and Medicaid programs, or knowingly making, using, or causing to be made or used a false record or statement material to a false or fraudulent claim or to avoid, decrease or conceal an obligation to pay money to the federal government. In addition, a claim for payment of items or services resulting from a violation of the federal Anti-Kickback Statute constitutes a false or fraudulent claim for purposes of the False Claims Act.

The civil monetary penalties statute imposes penalties against any person or entity who, among other things, is determined to have presented or caused to be presented a claim to a federal health program that the person knows or should know is for an item or service that was not provided as claimed or is false or fraudulent.

The federal Health Insurance Portability and Accountability Act of 1996 ("HIPAA"), created additional federal criminal statutes that prohibit knowingly and willfully executing, or attempting to execute, a scheme to defraud any healthcare benefit program or obtain, by means of false or fraudulent pretenses, representations or promises, any of the money or property owned by, or under the custody or control of, any healthcare benefit program, regardless of whether the payor is public or private, knowingly and willfully embezzling or stealing from a health care benefit program, willfully obstructing a criminal investigation of a health care offense and knowingly and willfully falsifying, concealing or covering up by any trick or device a material fact or making any materially false statements in connection with the delivery of, or payment for, healthcare benefits, items or services relating to healthcare matters. Similar to the federal Anti-Kickback Statute, a person or entity does not need to have actual knowledge of the statute, or the specific intent to violate it, to have committed a violation.

Additionally, the federal Physician Payments Sunshine Act and its implementing regulations, require certain manufacturers of drugs, devices, biologicals and medical supplies for which payment is available under Medicare, Medicaid or the Children's Health Insurance Program (with specified exceptions) to report annually information related to specified payments or other transfers of value provided to physicians (defined to include doctors, dentists, optometrists, podiatrists and chiropractors), certain other healthcare providers (physician assistants, nurse practitioners, clinical nurse specialists, certified nurse anesthetists, anesthesiologist assistants, and certified nurse-midwives) and teaching hospitals, or to entities or individuals at the request of, or designated on behalf of, such providers and teaching hospitals and to report annually specified ownership and investment interests held by physicians and their immediate family members.

Depending on the circumstances, failure to comply with these laws can result in significant penalties, including criminal, civil and/or administrative penalties, damages, fines, disgorgement, debarment from government contracts, individual imprisonment, additional reporting requirements and oversight if we become subject to a corporate integrity agreement or similar agreement to resolve allegations of non-compliance with these laws, exclusion from government programs, refusal to allow us to enter into supply contracts, including government contracts, reputational harm, diminished profits and future earnings and the curtailment or restructuring of our operations, any of which could adversely affect our business.

Data Privacy and Security Laws

We may be subject to data privacy and security laws, regulations, and standards by foreign, federal, state and local governments that govern the collection, use, access to, confidentiality and security of health-related and other personal information. In the United States, numerous federal and state laws and regulations, including data breach notification laws, health information privacy and security laws and consumer protection laws and regulations govern the collection, use, disclosure, and protection of health-related and other personal information. In addition, certain foreign laws govern the privacy and security of personal data, including health-related data. Privacy and security laws, regulations, and other obligations are constantly evolving, may conflict with each other to complicate compliance efforts, and can result in investigations, proceedings, or actions that lead to significant civil and/or criminal penalties and restrictions on data processing.

Healthcare Reform Measures

The United States and some foreign jurisdictions are considering or have enacted a number of legislative and regulatory proposals designed to change the healthcare system in ways that could affect our ability to sell our products profitably. Among policy makers and payors in the United States and elsewhere, there is significant interest in promoting changes in healthcare systems with the stated goals of containing healthcare costs, improving quality and/or expanding access. In the United States, the pharmaceutical industry has been a particular focus of these efforts and has been significantly affected by major legislative initiatives.

For example, in March 2010, the ACA was passed, which has substantially changed health care financing by both governmental and private insurers, and significantly affected the U.S. pharmaceutical industry. The ACA, among other things, subjected manufacturers to new annual fees and taxes for specified branded prescription drugs, increased the minimum Medicaid rebates owed by most manufacturers under the Medicaid Drug Rebate Program, expanded health care fraud and abuse laws, revised the methodology by which rebates owed by manufacturers to the state and federal government for covered outpatient drugs under the Medicaid Drug Rebate Program are calculated, imposed an inflation penalty on new formulations of drugs, extended the Medicaid Drug Rebate program to utilization of prescriptions of individuals enrolled in Medicaid managed care organizations, expanded the 340B program which caps the price at which manufacturers can sell covered outpatient pharmaceuticals to specified hospitals, clinics and community health centers, and provided incentives to programs that increase the federal government's comparative effectiveness research.

Since its enactment there have been executive, judicial and congressional challenges to certain aspects of the ACA. On June 17, 2021, the U.S. Supreme Court dismissed the most recent judicial challenge to the ACA brought by several states without specifically ruling on the constitutionality of the ACA. Prior to the Supreme Court's decision, President Biden issued an executive order to initiate a special enrollment period from February 15, 2021, through August 15, 2021, for purposes of obtaining health insurance coverage through the ACA marketplace. The executive order also instructed certain governmental agencies to review and reconsider their existing policies and rules that limit access to healthcare, including among others, reexamining Medicaid demonstration projects and waiver programs that include work requirements, and policies that create unnecessary barriers to obtaining access to health insurance coverage through Medicaid or the ACA.

Other legislative changes have been proposed and adopted in the United States since the ACA was enacted. In August 2011, the Budget Control Act of 2011, among other things, included aggregate reductions of Medicare payments to providers, which went into effect in April 2013, and, due to subsequent legislative amendments, will remain in effect through 2032 with the exception of a temporary suspension from May 1, 2020, through March 31, 2022, unless additional Congressional action is taken. In addition, in January 2013, the American Taxpayer Relief Act of 2012 was signed into law, which, among other things, reduced Medicare payments to several categories of healthcare providers and increased the statute of limitations period for the government to recover overpayments to providers from three to five years. On March 11, 2021, the American Rescue Plan Act of 2021 was signed into law, which eliminated the statutory Medicaid drug rebate cap, beginning January 1, 2024. The rebate was previously capped at 100% of a drug's average manufacturer price.

Further, there has been increasing legislative and enforcement interest in the United States with respect to specialty drug pricing practices. Specifically, there have been several recent U.S. Congressional inquiries and proposed and enacted federal and state legislation designed to, among other things, bring more transparency to drug pricing, review the relationship between pricing and manufacturer patient programs, reduce the cost of drugs under Medicare, and reform government program reimbursement methodologies for drugs. On August 16, 2022, the Inflation Reduction Act ("IRA"), of 2022 , was signed into law. Among other things, the IRA requires manufacturers of certain drugs to engage in price negotiations with Medicare (beginning in 2026), imposes rebates under Medicare Part B and Medicare Part D to penalize price increases that outpace inflation (first due in 2023), and replaces the Part D coverage gap discount program with a new discounting program (beginning in 2025). The IRA permits the Secretary of the Department of Health and Human Services, or HHS, to implement many of these provisions through guidance, as opposed to regulation, for the initial years. On August 29, 2023, HHS announced the list of the first ten drugs that will be subject to price negotiations, although the drug price negotiation program is currently subject to legal challenges. For that and other reasons, it is currently unclear how the IRA will be effectuated. At the state level, legislatures are increasingly passing legislation and implementing regulations designed to control pharmaceutical and biological product pricing, including price or patient reimbursement constraints, discounts, restrictions on certain product access and marketing cost disclosure and transparency measures, and, in some cases, designed to encourage importation from other countries and bulk purchasing.

We expect that additional state, federal and foreign healthcare reform measures will be adopted in the future, any of which could limit the amounts that federal, state and foreign governments will pay for healthcare product candidates and services, which could result in reduced demand for our products or additional pricing pressures.

On December 13, 2021, Regulation No 2021/2282 on HTA, amending Directive 2011/24/EU, was adopted. While the Regulation entered into force in January 2022, it will only begin to apply from January 2025 onwards, with preparatory and implementation-related steps to take place in the interim. Once applicable, it will have a phased implementation depending on the concerned products. The Regulation intends to boost cooperation among EU member states in assessing health technologies, including new medicinal products, and provide the basis for cooperation at the EU level for joint clinical assessments in these areas. It will permit EU member states to use common HTA tools, methodologies, and procedures across the EU, working together in four main areas, including joint clinical assessment of the innovative health technologies with the highest potential impact for patients, joint scientific consultations whereby developers can seek advice from HTA authorities, identification of emerging health technologies to identify promising technologies early, and continuing voluntary cooperation in other areas. Individual EU member states will continue to be responsible for assessing non-clinical (e.g., economic, social, ethical) aspects of health technology, and making decisions on pricing and reimbursement.

The Foreign Corrupt Practices Act

The Foreign Corrupt Practices Act ("FCPA") prohibits any U.S. individual or business from paying, offering or authorizing payment or offering of anything of value, directly or indirectly, to any foreign official, political party or candidate for the purpose of influencing any act or decision of the foreign entity in order to assist the individual or business in obtaining or retaining business. The FCPA also obligates companies whose securities are listed in the United States to comply with accounting provisions requiring the companies to maintain books and records that accurately and fairly reflect all transactions of the companies, including international subsidiaries, and to devise and maintain an adequate system of internal accounting controls for international operations.

Foreign Regulation

In addition to regulations in the United States, we will be subject to a variety of foreign regulations governing clinical trials and commercial sales and distribution of our products to the extent we choose to develop or sell any products outside of the United States. The foreign regulatory approval process includes all of the risks associated with FDA approval set forth above, as well as additional country-specific regulation.

Whether or not we obtain FDA approval for a product, we must obtain approval of a product by the comparable regulatory authorities of foreign countries before we can commence clinical trials or marketing of the product in those countries. Approval by one regulatory authority does not ensure approval by regulatory authorities in other jurisdictions. The approval process varies from country to country and the time may be longer or shorter than that required to obtain FDA approval. The requirements governing the conduct of clinical trials, product licensing, pricing and reimbursement vary greatly from country to country.

Non-clinical Studies and Clinical Trials

Similarly to the United States, the various phases of non-clinical and clinical research in the EU are subject to significant regulatory controls.

Non-clinical studies are performed to demonstrate the health or environmental safety of new chemical or biological substances. Non-clinical (pharmaco-toxicological) studies must be conducted in compliance with the principles of good laboratory practice ("GLP") as set forth in EU Directive 2004/10/EC (unless otherwise justified for certain particular medicinal products, e.g., radio pharmaceutical precursors for radio-labeling purposes). In particular, non-clinical studies, both in vitro and in vivo, must be planned, performed, monitored, recorded, reported and archived in accordance with the GLP principles, which define a set of rules and criteria for a quality system for the organizational process and the conditions for non-clinical studies. These GLP standards reflect the Organization for Economic Co-operation and Development requirements.

Clinical trials of medicinal products in the EU must be conducted in accordance with EU and national regulations and the International Council for Harmonization of Technical Requirements for Pharmaceuticals for Human Use ("ICH") guidelines on Good Clinical Practices ("GCP") as well as the applicable regulatory requirements and the ethical principles that have their origin in the Declaration of Helsinki.

If the sponsor of the clinical trial is not established within the EU, it must appoint an EU entity to act as its legal representative. The sponsor must take out a clinical trial insurance policy, and in most EU member states, the sponsor is liable to provide 'no fault' compensation to any study subject injured in the clinical trial.

The regulatory landscape related to clinical trials in the EU has been subject to recent changes. The EU Clinical Trials Regulation ("CTR") which was adopted in April 2014 and repeals the EU Clinical Trials Directive, became applicable on January 31, 2022. Unlike directives, the CTR is directly applicable in all EU member states without the need for member states to further implement it into national law. The CTR notably harmonizes the assessment and supervision processes for clinical trials throughout the EU via a Clinical Trials Information System, which contains a centralized EU portal and database.

While the EU Clinical Trials Directive required a separate clinical trial application ("CTA") to be submitted in each member state in which the clinical trial takes place, to both the competent national health authority and an independent ethics committee, much like the FDA and IRB respectively, the CTR introduces a centralized process and only requires the submission of a single application for multi-center trials. The CTR allows sponsors to make a single submission to both the competent authority and an ethics committee in each member state, leading to a single decision per member state. The CTA must include, among other things, a copy of the trial protocol and an investigational medicinal product dossier containing information about the manufacture and quality of the medicinal product under investigation. The assessment procedure of the CTA has been harmonized as well, including a joint assessment by all member states concerned, and a separate assessment by each member state with respect to specific requirements related to its own territory, including ethics rules. Each member state's decision is communicated to the sponsor via the centralized EU portal. Once the CTA is approved, clinical study development may proceed.

The CTR foresees a three-year transition period. The extent to which ongoing and new clinical trials will be governed by the CTR varies. Clinical trials for which an application was submitted (i) prior to January 31, 2022, under the EU Clinical Trials Directive, or (ii) between January 31, 2022, and January 31, 2023, and for which the sponsor has opted for the application of the EU Clinical Trials Directive remain governed by said Directive until January 31, 2025. After this date, all clinical trials (including those which are ongoing) will become subject to the provisions of the CTR.

Medicines used in clinical trials must be manufactured in accordance with Good Manufacturing Practice ("GMP"). Other national and EU-wide regulatory requirements may also apply.

Marketing Authorization

In order to market our product candidates in the EU and many other foreign jurisdictions, we must obtain separate regulatory approvals. More concretely, in the EU, medicinal product candidates can only be commercialized after obtaining a marketing authorization ("MA"). To obtain regulatory approval of a product candidate under EU regulatory systems, we must submit a MA application ("MAA"). The process for doing this depends, among other things, on the nature of the medicinal product. There are two types of MAs:

- "Centralized MAs" are issued by the European Commission through the centralized procedure based on the opinion of the Committee for Medicinal Products for Human Use ("CHMP") of the European Medicines Agency ("EMA") and are valid throughout the EU. The centralized procedure is compulsory for certain types of medicinal products such as (i) medicinal products derived from biotechnological processes, (ii) designated orphan medicinal products, (iii) advanced therapy medicinal products ("ATMPs") (such as gene therapy, somatic cell therapy and tissue engineered products) and (iv) medicinal products containing a new active substance indicated for the treatment of certain diseases, such as HIV/AIDS, cancer, diabetes, neurodegenerative diseases or autoimmune diseases and other immune dysfunctions, and viral diseases. The centralized procedure is optional for products containing a new active substance not yet authorized in the EU, or for products that constitute a significant therapeutic, scientific or technical innovation or which are in the interest of public health in the EU.

- "National MAs" are issued by the competent authorities of the EU member states, only cover their respective territory, and are available for product candidates not falling within the mandatory scope of the centralized procedure. Where a product has already been authorized for marketing in an EU member state, this national MA can be recognized in another member state through the mutual recognition procedure. If the product has not received a national MA in any member state at the time of application, it can be approved simultaneously in various member states through the decentralized procedure. Under the decentralized procedure an identical dossier is submitted to the competent authorities of each of the member states in which the MA is sought, one of which is selected by the applicant as the reference member state.

Under the above described procedures, in order to grant the MA, the EMA or the competent authorities of the EU member states make an assessment of the risk benefit balance of the product on the basis of scientific criteria concerning its quality, safety and efficacy. MAs have an initial duration of five years. After these five years, the authorization may be renewed on the basis of a reevaluation of the risk-benefit balance.

Data and Marketing Exclusivity

In the EU, new products authorized for marketing (i.e., reference products) generally receive eight years of data exclusivity and an additional two years of market exclusivity upon MA. If granted, the data exclusivity period prevents generic and biosimilar applicants from relying on the preclinical and clinical trial data contained in the dossier of the reference product when applying for a generic or biosimilar MA in the EU during a period of eight years from the date on which the reference product was first authorized in the EU. The market exclusivity period prevents a successful generic or biosimilar applicant from commercializing its product in the EU until ten years have elapsed from the initial MA of the reference product in the EU. The overall ten-year market exclusivity period can be extended to a maximum of eleven years if, during the first eight years of those ten years, the MA holder obtains an authorization for one or more new therapeutic indications, which, during the scientific evaluation prior to their authorization, are held to bring a significant clinical benefit in comparison with existing therapies. However, there is no guarantee that a product will be considered by the EU's regulatory authorities to be a new chemical or biological entity, and products may not qualify for data exclusivity.

Orphan Medicinal Products

The criteria for designating an "orphan medicinal product" in the EU are similar in principle to those in the United States. A medicinal product can be designated as an orphan if its sponsor can establish that: (1) the product is intended for the diagnosis, prevention or treatment of a life threatening or chronically debilitating condition (2) either (a) such condition affects not more than five in 10,000 persons in the EU when the application is made, or (b) the product, without the benefits derived from the orphan status, would not generate sufficient return in the EU to justify the necessary investment; and (3) there exists no satisfactory method of diagnosis, prevention or treatment of the condition in question that has been authorized for marketing in the EU or, if such method exists, the product will be of significant benefit to those affected by that condition.

Orphan designation must be requested before submitting an MAA. An EU orphan designation entitles a party to incentives such as reduction of fees or fee waivers, protocol assistance, and access to the centralized procedure. Upon grant of a MA, orphan medicinal products are entitled to ten years of market exclusivity for the approved indication, which means that the competent authorities cannot accept another MAA, or grant a MA, or accept an application to extend a MA for a similar medicinal product for the same indication for a period of ten years. The period of market exclusivity is extended by two years for orphan medicinal products that have also complied with an agreed pediatric investigation plan ("PIP"). No extension to any supplementary protection certificate can be granted on the basis of pediatric studies for orphan indications. Orphan designation does not convey any advantage in, or shorten the duration of, the regulatory review and approval process.

The orphan exclusivity period may be reduced to six years if, at the end of the fifth year, it is established that the product no longer meets the criteria for which it received orphan destination including where it is shown that the product is sufficiently profitable not to justify maintenance of market exclusivity or where the prevalence of the condition has increased above the threshold. Additionally, MA may be granted to a similar product for the same indication at any time if (i) the second applicant can establish that its product, although similar, is safer, more effective or otherwise clinically superior; (ii) the applicant consents to a second orphan medicinal product application; or (iii) the applicant cannot supply enough orphan medicinal product.

Pediatric Development

In the EU, MAAs for new medicinal products have to include the results of studies conducted in the pediatric population, in compliance with a PIP agreed with the EMA's Pediatric Committee ("PDCO"). The PIP sets out the timing and measures proposed to generate data to support a pediatric indication of the drug for which MA is being sought. The PDCO can grant a deferral of the obligation to implement some or all of the measures of the PIP until there are sufficient data to demonstrate the efficacy and safety of the product in adults. Further, the obligation to provide pediatric clinical trial data can be waived by the PDCO when these data is not needed or appropriate because the product is likely to be ineffective or unsafe in children, the disease or condition for which the product is intended occurs only in adult populations, or when the product does not represent a significant therapeutic benefit over existing treatments for pediatric patients. Once the MA is obtained in all the EU member states and study results are included in the product information, even when negative, the product is eligible for six months' supplementary protection certificate extension (if any is in effect at the time of approval) or, in the case of orphan pharmaceutical products, a two year extension of the orphan market exclusivity is granted.

Controlled substances

The EU legislation does not establish different classes of narcotic or psychotropic substances. However, the United Nations ("UN") Single Convention on Narcotic Drugs of 1961 and the UN Convention on Psychotropic Substances of 1971 ("UN Conventions") codify internationally applicable control measures to ensure the availability of narcotic drugs and psychotropic substances for medical and scientific purposes. The individual EU member states are all signatories to these UN Conventions. All signatories have a dual obligation to ensure that these substances are available for medical purposes and to protect populations against abuse and dependence. The UN Conventions regulate narcotic drugs and psychotropic substances as Schedule I, II, III, IV substances with Schedule II substances presenting the lowest relative risk of abuse among such substances and Schedule I and IV substances considered to present the highest risk of abuse.

The UN Conventions require signatories to require all persons manufacturing, trading (including exporting and importing) or distributing controlled substances to obtain a license from the relevant authority. Each individual export or import of a controlled substance must also be subject to an authorization. The obligations provided in the UN Conventions and additional requirements are implemented at national level and requirements may vary from one member state to another.

The aforementioned EU rules are generally applicable in the European Economic Area ("EEA") which consists of the 27 EU member states plus Norway, Liechtenstein and Iceland.

Failure to comply with EU and member state laws that apply to the conduct of clinical trials, manufacturing approval, MA of medicinal products and marketing of such products, both before and after grant of the MA, manufacturing of pharmaceutical products, statutory health insurance, bribery and anti-corruption or with other applicable regulatory requirements may result in administrative, civil or criminal penalties. These penalties could include delays or refusal to authorize the conduct of clinical trials, or to grant MA, product withdrawals and recalls, product seizures, suspension, withdrawal or variation of the MA, total or partial suspension of production, distribution, manufacturing or clinical trials, operating restrictions, injunctions, suspension of licenses, fines and criminal penalties.

Employees

As of December 31, 2023, we employed 69 employees, of which 65 were full-time employees.

Corporate Information

We were incorporated under the laws of the State of Iowa in October 2006, and were reincorporated under the laws of the State of Delaware in May 2014. We changed our name from KemPharm, Inc. to Zevra Therapeutics, Inc. effective as of February 21, 2023.

ITEM 1A. RISK FACTORS.

You should carefully consider all the risk factors and uncertainties described below, in addition to other information contained in this Annual Report on Form 10-K, including the section of this report titled "Management's Discussion and Analysis of Financial Condition and Results of Operations" and our financial statements and related notes, before investing in our common stock. If any of the following risks materialize, our business, financial condition and results of operations could be seriously harmed. This Annual Report on Form 10-K also contains forward-looking statements that involve risks and uncertainties. Our actual results could differ materially from those anticipated in the forward-looking statements because of factors that are described below and elsewhere in this Annual Report on Form 10-K.

Risks Related to the Development of Our Product Candidates

If commercialization of our approved products, or any of our product candidates, if approved, are not successful, or we experience significant delays in commercialization, our business will be harmed.

We currently generate minimal commercial revenue from the sale of our approved products and we may never be able to successfully commercialize a product candidate. We cannot guarantee that we, Corium, or any other collaborators will be able to successfully develop, manufacture or commercialize our approved products, or product candidates, if approved, or that we will ever receive any future payments under the AZSTARYS License Agreement. Despite the FDA's approval of OLPRUVA for oral suspension in the U.S. for the treatment of certain patients with UCDs, the product may not gain market acceptance among physicians, key opinion leaders, healthcare payors, patients and the medical community.

We have invested substantially all of our internal discovery and development efforts and much of our financial resources in the development of our proprietary LAT® platform technology, the identification of potential product candidates and the development of our product candidates. Our ability to generate revenue from our approved products and any of our product candidates, if approved, will depend heavily on their successful development and eventual commercialization. The success of our approved products and any of our product candidates will depend on several factors, including:

- successful completion of preclinical studies and requisite clinical trials;
- successful completion and achievement of endpoints in our clinical trials;
- demonstration that the risks involved with our approved products and any of our product candidates are outweighed by the benefits;
- successful development of our manufacturing processes for our approved products and for any of our product candidates, including entering into and maintaining arrangements with third-party manufacturers;
- successful completion of an FDA preapproval inspection of the facilities used to manufacture any of our product candidates for which we may submit an NDA;
- receipt of timely marketing approvals from applicable regulatory authorities, including, if applicable, the determination by the DEA of the controlled substance schedule for a product candidate, taking into account the recommendation of the FDA;
- obtaining differentiating claims in the labels for our product candidates;
- obtaining and maintaining patent, trademark and trade secret protection and regulatory exclusivity for our approved products and any of our product candidates and otherwise protecting our rights in our intellectual property portfolio;
- maintaining compliance with regulatory requirements, including cGMPs;
- launching commercial sales of our approved products, and any of our product candidates, if and when approved, whether alone or in collaboration with Corium or others;
- acceptance of our approved products and any of our product candidates, if approved, by patients, the medical community and third-party payors;
- competing effectively with other therapies;
- obtaining and maintaining healthcare coverage and adequate reimbursement; and
- maintaining a continued acceptable safety and efficacy profile of any of our products following approval.

Whether regulatory approval will be granted is unpredictable and depends upon numerous factors, including the substantial discretion of the regulatory authorities. If, following submission, our NDA or marketing authorization application for a product candidate is not accepted for substantive review or approval, the FDA or other comparable foreign regulatory authorities may require that we conduct additional studies or clinical trials, provide additional data, take additional manufacturing steps or require other conditions before they will reconsider our application. If the FDA or other comparable foreign regulatory authorities require additional studies, clinical trials or data, we would incur increased costs and delays in the marketing approval process, which may require us to expend more resources than we have available. In addition, the FDA or other comparable foreign regulatory authorities may not consider sufficient any additional required studies, clinical trials, data or information that we perform and complete or generate, or we may decide to abandon the program.

It is possible that none of our product candidates in clinical development or any of our future product candidates will ever obtain regulatory approval, even if we expend substantial time and resources seeking such approval.

If we do not achieve one or more of these factors in a timely manner or at all, we could experience significant delays, we could experience an inability to successfully commercialize any of our approved products or product candidates, if approved, or we could experience an inability to successfully commercialize any of our product candidates approved for marketing in the future, if any, which would harm our business.

Our research and development activities are focused on discovering and developing proprietary prodrugs, and we are taking an innovative approach to discovering and developing prodrugs, which may never lead to marketable prodrug products.

A key element of our strategy is to use our proprietary LAT platform technology to build a pipeline of prodrugs and progress product candidates based on these prodrugs through clinical development for the treatment of a variety of diseases and conditions. The scientific discoveries that form the basis for our efforts to discover and develop prodrugs are relatively new. As our scientific efforts are primarily focused on discovering novel prodrugs with new molecular structures, the evidence to support the feasibility of developing product candidates based on these discoveries is both preliminary and limited. Although our research and development efforts to date have resulted in a pipeline of prodrug product candidates, we may not be able to develop those product candidates into prodrugs that are bioequivalent, safe and/or effective or that offer commercially significant improvements over already approved drugs. Even if we are successful in continuing to build our pipeline, the potential product candidates that we identify may not be suitable for clinical development, for reasons including being shown to have harmful side effects, a lack of efficacy, or other characteristics that indicate that they are unlikely to be prodrugs that will receive marketing approval and achieve market acceptance. If our approved products are not successfully commercialized and we do not successfully develop and commercialize any of our product candidates based upon our proprietary LAT platform technology, we will not be able to obtain product revenue in future periods, which likely would result in significant harm to our financial position and adversely affect our stock price.

If we are not able to obtain required regulatory approvals for any of our product candidates, or the approved labels are not sufficiently differentiated from other competing products, we will not be able to commercialize them and our ability to generate revenue or profits or to raise future capital could be limited.

The research, testing, manufacturing, labeling, packaging, storage, approval, sale, marketing, advertising and promotion, pricing, export, import and distribution of drug products are subject to extensive regulation by the FDA and other regulatory authorities in the United States and other countries, which regulations differ from country to country and change over time. We are not permitted to market any of our product candidates in the United States until we receive approval of an NDA from the FDA, or in any foreign countries until we receive the requisite approvals in such countries. In the United States, the FDA generally requires the completion of non-clinical testing and clinical trials of each drug to establish its safety and efficacy and extensive pharmaceutical development to ensure its quality and other factors before an NDA is approved. Regulatory authorities in other jurisdictions impose similar requirements. Of the large number of drugs in development, only a small percentage result in the submission of an NDA to the FDA and even fewer are approved for commercialization.

Even if regulatory approval is obtained, subsequent safety, efficacy, quality or other issues can result in a product approval being suspended or withdrawn, or the approved label for any approved product may not be sufficiently differentiated from other competing products to support market adoption thereof. In March 2021, the FDA approved the NDA for AZSTARYS and in December 2022, the FDA approved OLPRUVA. Even with the regulatory approval of AZSTARYS and OLPRUVA by the FDA, we cannot guarantee that the FDA will approve any of our product candidates for commercial sale or approve any proposed label we may have for any such product candidate. If our development efforts for our product candidates, including our efforts to obtain regulatory approval, are not successful for their planned indications or are delayed, or if adequate demand for our product candidates that are approved for marketing, if any, is not generated, our business will be harmed.

The success of our product candidates will depend on the receipt and maintenance of regulatory approval and the issuance and maintenance of such approval is uncertain and subject to a number of risks, including the following:

- the FDA or comparable foreign regulatory authorities, institutional review boards, or IRBs, or ethics committees may disagree with the design or conduct of our clinical trials;
- the results of our clinical trials may not meet the level of statistical or clinical significance required by the FDA or other regulatory agencies for marketing approval or for us to receive approval for claims that are necessary for commercialization;
- the dosing in a particular clinical trial may not be at an optimal level;
- patients in our clinical trials may suffer adverse effects for reasons that may or may not be related to our product candidates;
- the data collected from clinical trials may not be sufficient to support submissions to regulatory authorities or to obtain regulatory approval in the United States or elsewhere;
- the FDA or comparable foreign regulatory authorities may fail to approve the manufacturing processes or facilities of third-party manufacturers with which we contract for clinical and commercial supplies or may later suspend or withdraw such approval;
- the approval policies or regulations of the FDA or comparable foreign regulatory authorities may significantly change in a manner rendering our clinical data insufficient for approval; and
- even if we obtain marketing approval in one or more countries, future safety or other issues could result in the suspension or withdrawal of regulatory approval in such countries.

We have only limited experience in submitting the applications necessary to gain regulatory approvals and have relied, and expect to continue to rely, on consultants and third-party contract research organizations, or CROs, with expertise in this area to assist us in this process. Securing FDA approval requires the submission of extensive non-clinical and clinical data, information about product manufacturing processes and inspection of facilities and supporting information to the FDA for each therapeutic indication to establish a product candidate's safety and efficacy for each indication and manufacturing quality. Additionally, we cannot guarantee that regulators will agree with our assessment of the results of the clinical trials we have conducted or that any future trials will be successful.

Any product candidates we develop may prove to have undesirable or unintended side effects, toxicities or other characteristics that may preclude our obtaining regulatory approval or prevent or limit commercial use with respect to one or all intended indications.

The process of obtaining regulatory approvals is expensive, often takes many years, if approval is obtained at all, and can vary substantially based upon, among other things, the type, complexity and novelty of the product candidates involved, the jurisdiction in which regulatory approval is sought and the substantial discretion of the regulatory authorities. Changes in the regulatory approval policy during the development period, changes in or the enactment of additional statutes or regulations, or changes in regulatory review for a submitted product application may cause delays in the approval or rejection of an application or may result in future withdrawal of approval. Regulatory approval obtained in one jurisdiction does not necessarily mean that a product candidate will receive regulatory approval in all jurisdictions in which we may seek approval, but the failure to obtain approval in one jurisdiction may negatively impact our ability to seek approval in a different jurisdiction. Failure to obtain regulatory marketing approval of our product candidates in any indication will prevent us from commercializing those product candidates for that indication, and our ability to generate revenue will be impaired.

Disruptions at the FDA and other government agencies caused by funding shortages or global health concerns could hinder their ability to hire, retain or deploy key leadership and other personnel, or otherwise prevent new or modified products from being developed, approved or commercialized in a timely manner or at all, which could negatively impact our business.

The ability of the FDA and foreign regulatory authorities to review and or approve new products can be affected by a variety of factors, including government budget and funding levels, statutory, regulatory, and policy changes, the FDA's and foreign regulatory authorities' ability to hire and retain key personnel and accept the payment of user fees, and other events that may otherwise affect the FDA's ability to perform routine functions. Average review times at the FDA and foreign regulatory authorities have fluctuated in recent years as a result. In addition, government funding of other government agencies that fund research and development activities is subject to the political process, which is inherently fluid and unpredictable. Disruptions at the FDA and other agencies may also slow the time necessary for new drugs to be reviewed and/or approved by necessary government agencies, which would adversely affect our business. For example, over the last several years, the U.S. government has shut down several times and certain regulatory agencies, such as the FDA, have had to furlough critical FDA employees and stop critical activities.

In response to the COVID-19 pandemic, the FDA announced its intention to postpone most inspections of foreign and domestic manufacturing facilities at various points. Even though the FDA has since resumed standard inspection operations of domestic facilities where feasible, the FDA has continued to monitor and implement changes to its inspectional activities to ensure the safety of its employees and those of the firms it regulates as it adapts to the evolving COVID-19 pandemic, and any resurgence of the virus or emergence of new variants may lead to further inspection delays. Regulatory authorities outside the United States have adopted similar restrictions or other policy measures in response to the COVID-19 pandemic. If a prolonged government shutdown occurs, or if global health concerns continue to prevent the FDA or other regulatory authorities from conducting their regular inspections, reviews, or other regulatory activities, it could significantly impact the ability of the FDA or other regulatory authorities to timely review and process our regulatory submissions, which could have a material adverse effect on our business.

If the FDA does not conclude that our product candidates satisfy the requirements for the 505(b)(2) NDA pathway as anticipated, the approval pathway for our product candidates will likely take significantly longer, cost significantly more and entail significantly greater complications and risks than anticipated, and the FDA may not ultimately approve our product candidates.

A key element of our strategy is to seek FDA approval for most of our product candidates under Section 505(b)(2) of the FFDCA, otherwise known as the 505(b)(2) NDA pathway with any NDA submitted thereunder a 505(b)(2) NDA, where possible. The 505(b)(2) NDA pathway permits the filing of an NDA where at least some of the information required for approval comes from studies not conducted by or for the applicant and for which the applicant has not obtained a right of reference. Section 505(b)(2), if applicable to us under the FFDCA, would allow an NDA we submit to the FDA to rely in part on data in the public domain or the FDA's prior conclusions regarding the safety and effectiveness of approved compounds, which could expedite the development program for our future product candidates by potentially decreasing the amount of nonclinical and/or clinical data that we would need to generate in order to obtain FDA approval. Such reliance is typically predicated on a showing of bioequivalence or comparable bioavailability to an approved drug. AZSTARYS was approved via the 505(b)(2) NDA pathway on March 2, 2021.

If the FDA does not allow us to pursue the 505(b)(2) NDA pathway as anticipated, or if we cannot demonstrate bioequivalence or comparable bioavailability of our product candidates to approved products, we may need to conduct additional clinical trials, provide additional data and information, and meet additional standards for regulatory approval. Moreover, even if the FDA does allow us to pursue the 505(b)(2) NDA pathway, depending on the product candidate, we may still need to conduct additional clinical trials, including clinical trials to assess product safety or efficacy. If this were to occur, the time and financial resources required to obtain FDA approval for our product candidates, and complications and risks associated with our product candidates, would likely substantially increase.

Moreover, our inability to pursue the 505(b)(2) NDA pathway could result in new competitive products reaching the market more quickly than our product candidates, which could hurt our competitive position and our business prospects. Even if we are allowed to pursue the 505(b)(2) NDA pathway, we cannot assure you that our product candidates will receive the requisite approvals for commercialization on a timely basis, if at all. Other companies may achieve product approval of similar products before we do, which would delay our ability to obtain product approval, expose us to greater competition, and would require that we seek approval via alternative pathways.

In addition, notwithstanding the approval of several products by the FDA under Section 505(b)(2) over the last few years, pharmaceutical companies and others have objected to the FDA's interpretation of Section 505(b)(2). If the FDA's interpretation of Section 505(b)(2) is successfully challenged, the FDA may change its policies and practices with respect to Section 505(b)(2) regulatory approvals, which could delay or even prevent the FDA from approving any NDA that we submit under Section 505(b)(2). In addition, the pharmaceutical industry is highly competitive, and Section 505(b)(2) NDAs are subject to certain requirements designed to protect the patent rights of sponsors of previously approved drugs that are referenced in a Section 505(b)(2) NDA. These requirements may give rise to patent litigation and mandatory delays in approval of our NDAs for up to 30 months or longer depending on the outcome of any litigation. It is not uncommon for a manufacturer of an approved product to file a citizen petition with the FDA seeking to delay approval of, or impose additional approval requirements for, pending competing products. If successful, such petitions can significantly delay, or even prevent, the approval of a new product. Even if the FDA ultimately denies such a petition, the FDA may substantially delay approval while it considers and responds to the petition. In addition, even if we are able to utilize the Section 505(b)(2) regulatory pathway, there is no guarantee this would ultimately lead to streamlined product development or earlier approval.

Clinical drug development involves a lengthy and expensive process, with an uncertain outcome. We may incur additional costs or experience delays in completing, or ultimately be unable to complete, the development and commercialization of our product candidates.

The risk of failure for our product candidates is high. It is impossible to predict when or if any of our current product candidates will prove effective or safe in humans and will receive regulatory approval. Before obtaining marketing approval from regulatory authorities for the sale of any product candidate, we must complete preclinical development and then conduct clinical trials to demonstrate the safety and efficacy of our product candidates in humans. Clinical testing is expensive, difficult to design and implement, can take many years to complete and is uncertain as to outcome. A failure of one or more clinical trials can occur at any stage of testing. The outcome of preclinical studies and early clinical trials may not be predictive of the success of later clinical trials, and interim results of a clinical trial do not necessarily predict final results. Interpretation of results from early, usually smaller, studies that suggest positive trends in some subjects, requires caution. Results from later stages of clinical trials enrolling more subjects may fail to show the desired safety and efficacy results or otherwise fail to be consistent with the results of earlier trials of the same product candidates. Later clinical trial results may not replicate earlier clinical trials for a variety of reasons, including differences in trial design, different trial endpoints, or lack of trial endpoints in exploratory studies, subject population, number of subjects, subject selection criteria, trial duration, drug dosage and formulation and lack of statistical power in the earlier studies. Moreover, preclinical and clinical data are often susceptible to varying interpretations and analyses, and many companies that have believed their product candidates performed satisfactorily in preclinical studies and clinical trials have nonetheless failed to obtain marketing approval of their products.

We may experience numerous unforeseen events during, or as a result of, clinical trials that could delay or prevent our ability to receive marketing approval or commercialize our product candidates, including:

- regulators, IRBs, or other ethics committees may not authorize us or our investigators to commence a clinical trial, conduct a clinical trial at a prospective trial site or amend clinical trial protocols as needed;
- we may experience delays in reaching, or fail to reach, agreement on acceptable clinical trial contracts or clinical trial protocols with prospective trial sites and CROs;
- clinical trials of our product candidates may produce negative or inconclusive results, including failure to demonstrate statistical significance in cases where that is required, and we may decide, or regulators may require us, to conduct additional clinical trials or abandon prodrug development programs;
- we may be unable to obtain sufficient or adequate supply or quality of product candidates or other materials necessary for use in clinical trials, or experience delays in sufficiently developing, characterizing or controlling a manufacturing process suitable for clinical trials;
- we may experience delays in manufacturing, testing, releasing, validating or importing/exporting sufficient stable quantities of our product candidates for use in clinical trials or the inability to do any of the foregoing;
- the number of subjects required for clinical trials of our product candidates may be larger than we anticipate enrollment in these clinical trials may be slower than we anticipate, or participants may drop out of these clinical trials at a higher rate than we anticipate;
- our third-party contractors may fail to comply with regulatory requirements or trial protocols, or meet their contractual obligations to us in a timely manner, or at all;
- regulators, IRBs, or other ethics committees may require that we or our investigators suspend or terminate clinical research for various reasons, including noncompliance with regulatory requirements or a finding that the participants are being exposed to unacceptable health risks;
- the cost of clinical trials of our product candidates may be greater than we anticipate, including if we are not able to pursue the 505(b)(2) NDA pathway for approval of our product candidates;
- we will need to pay substantial application user fees, which we may not be able to afford;
- we may be required to transfer manufacturing processes to larger-scale facilities operated by a contract manufacturing organization, and we may experience delays or failures by our contract manufacturers to make any necessary changes to such manufacturing process;
- we may abandon our development program or programs based on the changing regulatory or commercial environment;
- regulatory authorities may not agree with our trial design or implementation; and
- our product candidates may have undesirable side effects or other unexpected characteristics, causing us or our investigators, regulators or IRBs to suspend or terminate the trials.

If we are required to conduct additional clinical trials or other testing of our product candidates beyond those that we currently contemplate, if we are unable to successfully complete clinical trials of our product candidates or other testing, if the results of these trials or tests are not positive or are only modestly positive or if there are safety concerns, we may:

- be delayed in obtaining marketing approval for our product candidates;
- not obtain marketing approval at all;
- obtain approval for indications or patient populations that are not as broad as intended or desired;
- obtain approval but without the claims necessary for us to successfully commercialize our product candidates;
- obtain approval with labeling that includes significant use or distribution restrictions or safety warnings;
- be subject to additional post-marketing testing, surveillance, or other requirements, such as REMS; or
- have the product removed from the market after obtaining marketing approval.

Any inability to successfully initiate or complete clinical trials could result in additional costs to us or impair our ability to generate revenue from product sales. In addition, if we make manufacturing or formulation changes to our product candidates, we may be required to or we may elect to conduct additional studies to bridge our modified product candidates to earlier versions. Clinical trial delays could also shorten any periods during which our products have patent protection and may allow our competitors to bring products to market before we do, which could impair our ability to successfully commercialize our product candidates and may seriously harm our business.

Clinical trials must be conducted in accordance with the FDA and other applicable regulatory authorities' legal requirements, regulations or guidelines, and are subject to oversight by these governmental agencies and ethics committees or IRBs at the medical institutions where the clinical trials are conducted. We could encounter delays if a clinical trial is suspended or terminated by us, by the data safety monitoring board for such trial or by the FDA or any other regulatory authority, or if the IRBs of the institutions in which such trials are being conducted suspend or terminate the participation of their clinical investigators and sites subject to their review. Such authorities may suspend or terminate a clinical trial due to a number of factors, including failure to conduct the clinical trial in accordance with regulatory requirements or our clinical protocols, inspection of the clinical trial operations or trial site by the FDA or other regulatory authorities resulting in the imposition of a clinical hold, unforeseen safety issues or adverse side effects, failure to demonstrate a benefit from using a product candidate, changes in governmental regulations or administrative actions or lack of adequate funding to continue the clinical trial.

Further, conducting clinical trials in foreign countries, as we may do for our product candidates, presents additional risks that may delay completion of our clinical trials. These risks include the failure of enrolled patients in foreign countries to adhere to clinical protocol as a result of differences in healthcare services or cultural customs, managing additional administrative burdens associated with foreign regulatory schemes, as well as political and economic risks relevant to such foreign countries.

Moreover, principal investigators for our clinical trials may serve as scientific advisors or consultants to us from time to time and receive compensation in connection with such services. Under certain circumstances, we may be required to report some of these relationships to the FDA or comparable foreign regulatory authorities. The FDA or comparable foreign regulatory authority may conclude that a financial relationship between us and a principal investigator has created a conflict of interest or otherwise affected interpretation of the study. The FDA or comparable foreign regulatory authority may therefore question the integrity of the data generated at the applicable clinical trial site and the utility of the clinical trial itself may be jeopardized. This could result in a delay in approval, or rejection, of our marketing applications by the FDA or comparable foreign regulatory authority, as the case may be, and may ultimately lead to the denial of marketing approval of one or more of our product candidates.

Delays in the completion of any clinical trial of our product candidates will increase our costs, slow down our product development and the regulatory approval processes and delay or potentially jeopardize our ability to commence product sales and generate product revenue. Any of these occurrences may harm our business, financial condition and prospects significantly.

In addition, the FDA's and other regulatory authorities' policies with respect to clinical trials may change and additional government regulations may be enacted. For instance, the regulatory landscape related to clinical trials in the EU recently evolved. The EU Clinical Trials Regulation ("CTR") which was adopted in April 2014 and repeals the EU Clinical Trials Directive, became applicable on January 31, 2022. While the EU Clinical Trials Directive required a separate clinical trial application ("CTA") to be submitted in each member state in which the clinical trial takes place, to both the competent national health authority and an independent ethics committee, the CTR introduces a centralized process and only requires the submission of a single application for multi-center trials. The CTR allows sponsors to make a single submission to both the competent authority and an ethics committee in each member state, leading to a single decision per member state. The assessment procedure of the CTA has been harmonized as well, including a joint assessment by all member states concerned, and a separate assessment by each member state with respect to specific requirements related to its own territory, including ethics rules. Each member state's decision is communicated to the sponsor via the centralized EU portal. Once the CTA is approved, clinical study development may proceed. The CTR foresees a three-year transition period. The extent to which ongoing and new clinical trials will be governed by the CTR varies. Clinical trials for which an application was submitted (i)prior to January 31, 2022, under the Clinical Trials Directive, or (ii) between January 31, 2022, and January 31, 2023, and for which the sponsor has opted for the application of the EU Clinical Trials Directive remain governed by said Directive until January 31, 2025. After this date, all clinical trials (including those which are ongoing) will become subject to the provisions of the CTR. Compliance with the CTR requirements by us and our third-party service providers, such as CROs, may impact our developments plans.

If we experience delays or difficulties in the enrollment of subjects in clinical trials, our receipt of necessary regulatory approvals could be delayed or prevented.

We may not be able to initiate or continue clinical trials for our product candidates if we are unable to locate and enroll a sufficient number of eligible subjects to participate in these trials as may be required by the FDA or similar regulatory authorities outside the United States. We cannot predict how successful we will be at enrolling subjects in future clinical trials. If we are not successful at enrolling subjects in one clinical trial, it may affect when we are able to initiate our next clinical trial, which could result in significant delays in our efforts to pursue regulatory approval of and commercialize our product candidates. In addition, some of our competitors have ongoing clinical trials to treat the same indications as our product candidates, and subjects who would otherwise be eligible for our clinical trials may instead enroll in clinical trials of our competitors. Subject enrollment is affected by other factors including:

- the size and nature of the subject population specified in the trial protocol;
- the eligibility criteria for the study in question;
- the perceived risks and benefits of the product candidate under study;
- the fact that the product candidate is a controlled substance;
- severe or unexpected drug-related adverse events experienced by subjects in a clinical trial;
- the availability of drugs approved to treat the diseases or conditions under study;
- the efforts to facilitate timely enrollment in clinical trials;
- the patient referral practices of physicians;
- the severity of the disease or condition under investigation;
- the ability to obtain and maintain subject informed consent;
- the ability to retain subjects in the clinical trial and their return for follow-up;
- the clinical trial design, including required tests, procedures and follow-up;
- the ability to monitor subjects adequately during and after treatment;
- delays in adding new investigators and clinical sites;
- withdrawal of clinical trial sites from clinical trials; and
- the proximity and availability of clinical trial sites for prospective subjects.

Our inability to enroll a sufficient number of subjects for clinical trials would result in significant delays and could require us to abandon one or more clinical trials altogether. Enrollment delays in these clinical trials may result in increased development costs for our product candidates, which could cause our value to decline and limit our ability to obtain additional financing.

Interim, topline, or preliminary data from our clinical trials that we announce or publish from time to time may change as more patient data become available and are subject to audit and verification procedures that could result in material changes in the final data.

From time to time, we may publicly disclose topline or preliminary data from our preclinical studies and clinical trials, which is based on a preliminary analysis of then-available data, and the results and related findings and conclusions are subject to change following a more comprehensive review of the data related to the particular study or trial. We also make assumptions, estimations, calculations, and conclusions as part of our analyses of data, and we may not have received or had the opportunity to fully and carefully evaluate all data. As a result, the preliminary or topline results that we report may differ from future results of the same studies, or different conclusions or considerations may qualify such results, once additional data has been received and fully evaluated. Topline data also remains subject to audit and verification procedures that may result in the final data being materially different from the preliminary data we previously published. As a result, preliminary or topline data should be viewed with caution until the final data are available.

From time to time, we may also disclose interim data from our preclinical studies and clinical trials. Interim data from clinical trials that we may complete are subject to the risk that one or more of the clinical outcomes may materially change as patient enrollment continues and more patient data become available or as patients from our clinical trials continue other treatments for their disease. Adverse differences between preliminary or interim data and final data could significantly harm our business prospects.

Further, disclosure of interim data by us or by our competitors could result in volatility in the price of our common stock. Further, others, including regulatory agencies, may not accept or agree with our assumptions, estimates, calculations, conclusions, or analyses or may interpret or weigh the importance of data differently, which could impact the value of the particular program, the approvability or commercialization of the particular product candidate, and our company in general. If the interim, topline, or preliminary data that we report differs from actual results, or if others, including regulatory authorities, disagree with the conclusions reached, our ability to obtain approval for, and commercialize, any of our potential product candidates may be harmed, which could harm our business, operating results, prospects, or financial condition.

We may not be able to obtain or maintain orphan drug designations which we pursue for any of our product candidates, and we may be unable to maintain the benefits associated with orphan drug designation, including the potential for market exclusivity.

Regulatory authorities in some jurisdictions, including the United States, may designate drugs for relatively small patient populations as orphan drugs. Under the Orphan Drug Act of 1983, the FDA may designate a drug as an orphan product if it is intended to treat a rare disease or condition, which is generally defined as a patient population of fewer than 200,000 individuals in the United States, or a patient population of greater than 200,000 individuals in the United States, but for which there is no reasonable expectation that the cost of developing the drug will be recovered from sales in the United States. While the FDA has granted KP1077 orphan drug designation for the treatment of IH, and celiprolol received orphan drug designation from the FDA for the treatment of vEDS, we have not received orphan drug designation for any other product candidate. We may seek to obtain orphan drug designation for product but there can be no assurance that the FDA will grant orphan designation for any indication for which we apply, or that we will be able to maintain such designation.

In the United States, orphan designation entitles a party to financial incentives such as opportunities for grant funding toward clinical trial costs, tax advantages and user-fee waivers. In addition, if a product candidate that has orphan designation subsequently receives the first FDA approval for the disease for which it has such designation, the product is entitled to orphan drug exclusivity, which means that the FDA may not approve any other applications, including an NDA, to market the same product for the same indication for seven years, except in limited circumstances, such as a showing of clinical superiority to the product with orphan drug exclusivity or where the manufacturer is unable to assure sufficient product quantity.

In the EU, orphan designation is granted by the EC based on a scientific opinion of the EMA's Committee for Orphan Medicinal Products. A medicinal product may be designated as orphan if its sponsor can establish that (i) the product is intended for the diagnosis, prevention or treatment of a life-threatening or chronically debilitating condition; (ii) either (a) such condition affects no more than 5 in 10,000 persons in the EU when the application is made, or (b) the product, without the benefits derived from orphan status, would not generate sufficient return in the EU to justify investment; and (iii) there exists no satisfactory method of diagnosis, prevention or treatment of such condition authorized for marketing in the EU, or if such a method exists, the medicinal product will be of significant benefit to those affected by the condition. The application for orphan designation must be submitted before the application for marketing authorization. Orphan designation entitles a party to financial incentives such as reduction of fees, fee waivers, protocol assistance, and access to the centralized marketing authorization procedure. Moreover, upon grant of a marketing authorization and assuming the requirement for orphan designation are also met at the time the marketing authorization is granted, orphan medicinal products are entitled to a ten-year period of market exclusivity for the approved therapeutic indication. The period of market exclusivity is extended by two years for orphan medicinal products that have also complied with an agreed Pediatric Investigation Plan.

Even if we obtain orphan drug exclusivity for a product, that exclusivity may not effectively protect the product from competition because different drugs can be approved for the same condition. Even after an orphan drug is approved, the FDA or comparable foreign regulatory authority can subsequently approve the same drug for the same condition if such regulatory authority concludes that the later drug is clinically superior because it is shown to be safer, more effective or makes a major contribution to patient care.

Orphan drug exclusivity may also be lost if the FDA later determines that the initial request for designation was materially defective. In addition, orphan drug exclusivity does not prevent the FDA from approving competing drugs for the same or similar indication containing a different active ingredient. In addition, if a subsequent drug is approved for marketing for the same or a similar indication as any of our product candidates that receive marketing approval, we may face increased competition and lose market share regardless of orphan drug exclusivity. In the EU, during the market exclusivity period, marketing authorizations may be granted to a similar medicinal product with the same orphan indication if: (i) the applicant can establish that the second medicinal product, although similar to the orphan medicinal product already authorized is safer, more effective or otherwise clinically superior to the orphan medicinal product already authorized; (ii) the marketing authorization holder for the orphan medicinal product grants its consent; or (iii) if the marketing authorization holder of the orphan medicinal product is unable to supply sufficient quantities of product. The European exclusivity period can be reduced to six years, if, at the end of the fifth year a medicine no longer meets the criteria for orphan designation (i.e. the prevalence of the condition has increased above the orphan designation threshold or it is judged that the product is sufficiently profitable so as not to justify maintenance of market exclusivity).

Orphan drug designation neither shortens the development time or regulatory review time of a drug nor gives the drug any advantage in the regulatory review or approval process.

Our approved products and certain of our product candidates contain controlled substances, the manufacture, use, sale, importation, exportation, prescribing and distribution of which are subject to regulation by the DEA and other regulatory agencies.

Our approved products and certain of our product candidates are regulated as controlled substances, which are subject to state, federal, and foreign laws and regulations regarding their manufacture, use, sale, importation, exportation, and distribution. Among other things, controlled substances are regulated under the federal Controlled Substances Act of 1970, or CSA, and regulations of the DEA. Before we can commercialize any of our products or product candidates, if approved, the DEA will need to determine the controlled substance schedule, taking into account the recommendation of the FDA. This may be a lengthy process that could delay our marketing of a product candidate and could potentially diminish any regulatory exclusivity periods for which we may be eligible.

The DEA regulates controlled substances as Schedule I, II, III, IV or V substances. Schedule I substances by definition have no established medicinal use and may not be marketed or sold in the United States. A pharmaceutical product may be listed as Schedule II, III, IV or V, with Schedule II substances considered to present the highest risk of abuse and Schedule V substances the lowest relative risk of abuse among such substances. The DEA determined AZSTARYS to be a Schedule II controlled substance. In addition, the DEA scheduled SDX as a Schedule IV substance. We expect that most of our product candidates, including KP1077, if approved, will also be regulated as "controlled substances" by the DEA, which subjects AZSTARYS and this product candidate to additional restrictions regarding their manufacture, shipment, storage, sale and use, depending on the scheduling of the active ingredients, and may limit the commercial potential of our products and any of our product candidates, if approved.

Various states also independently regulate controlled substances. Though state-controlled substances laws often mirror federal law, because the states are separate jurisdictions, they may separately schedule drugs as well. While some states automatically schedule a drug when the DEA does so, in other states there must be rulemaking or a legislative action. State scheduling may delay commercial sale of any controlled substance drug product for which we obtain federal regulatory approval and adverse scheduling could impair the commercial attractiveness of such product. We or our collaborators must also obtain separate state registrations in order to be able to obtain, handle and distribute controlled substances for clinical trials or commercial sale, and failure to meet applicable regulatory requirements could lead to enforcement and sanctions from the states in addition to those from the DEA or otherwise arising under federal law.

For any of our products or product candidates classified as controlled substances, we and our suppliers, manufacturers, contractors, customers and distributors are required to obtain and maintain applicable registrations from state, federal and foreign law enforcement and regulatory agencies and comply with state, federal and foreign laws and regulations regarding the manufacture, use, sale, importation, exportation and distribution of controlled substances. There is a risk that DEA regulations may limit the supply of the compounds used in clinical trials for our product candidates, and, in the future, the ability to produce and distribute our products in the volume needed to meet commercial demand. For example, a DEA quota system controls and limits the availability and production of controlled substances in Schedule I or II. Because our products and most of our product candidates are or may be regulated as Schedule II controlled substances, they may be subject to the DEA's production and procurement quota scheme. The DEA establishes annually an aggregate quota for how much of a controlled substance may be produced in total in the United States based on the DEA's estimate of the quantity needed to meet legitimate scientific and medicinal needs. Manufacturers of Schedule I and II controlled substances are required to apply for quotas on an annual basis. If we or our contract manufacturers or suppliers do not obtain a sufficient quota from the DEA, we may not be able to obtain sufficient quantities of these controlled substances in order to complete our clinical trials or meet commercial demand for any of our products or product candidates classified under Schedule II.

Regulations associated with controlled substances govern manufacturing, labeling, packaging, testing, dispensing, production and procurement quotas, recordkeeping, reporting, handling, shipment and disposal. These regulations increase the personnel needs and the expense associated with development and commercialization of product candidates including controlled substances. The DEA, and some states, conduct periodic inspections of registered establishments that handle controlled substances. Failure to obtain and maintain required registrations or comply with any applicable regulations could delay or preclude us from developing and commercializing our product candidates containing controlled substances and subject us to enforcement action. The DEA may seek civil penalties, refuse to renew necessary registrations or initiate proceedings to revoke those registrations. In some circumstances, violations could lead to criminal proceedings. Because of their restrictive nature, these regulations could limit commercialization of any of our products or product candidates that are classified as controlled substances.

The EU legislation does not establish different classes of narcotic or psychotropic substances. However, the United Nations ("UN") Single Convention on Narcotic Drugs of 1961 and the UN Convention on Psychotropic Substances of 1971 ("UN Conventions") codify internationally applicable control measures to ensure the availability of narcotic drugs and psychotropic substances for medical and scientific purposes. The individual EU member states are all signatories to these UN Conventions. All signatories have a dual obligation to ensure that these substances are available for medical purposes and to protect populations against abuse and dependence. The UN Conventions regulate narcotic drugs and psychotropic substances as Schedule I, II, III, IV substances with Schedule II substances presenting the lowest relative risk of abuse among such substances and Schedule I and IV substances considered to present the highest risk of abuse.

The UN Conventions require signatories to require all persons manufacturing, trading (including exporting and importing) or distributing controlled substances to obtain a license from the relevant authority. Each individual export or import of a controlled substance must also be subject to an authorization. The obligations provided in the UN Conventions and additional requirements are implemented at national level and requirements may vary from one member state to another. In order to develop and commercialize our products in the EU, we would need to comply with the national requirements related to controlled substances which is costly and may affect our development plans in the EU.

Our products and product candidates may be associated with serious adverse events, undesirable side effects or have other properties that could halt their clinical development, prevent their regulatory approval, limit their commercial potential or result in significant negative consequences.

Adverse events or other undesirable side effects caused by our products or product candidates could cause us or regulatory authorities to interrupt, delay or halt clinical trials and could result in a more restrictive label or the delay or denial of regulatory approvals by the FDA or other comparable foreign regulatory authorities.

During the conduct of clinical trials, patients report changes in their health, including illnesses, injuries, and discomforts, to their study doctor. Often, it is not possible to determine whether or not the product candidate being studied caused these conditions. It is possible that as we test our product candidates in larger, longer and more extensive clinical trials, or as use of these product candidates becomes more widespread if they receive regulatory approval, illnesses, injuries, discomforts and other adverse events that were observed in previous trials, as well as conditions that did not occur or went undetected in previous trials, will be reported by patients. Many times, side effects are only detectable after investigational products are tested in large-scale clinical trials or, in some cases, after they are made available to patients on a commercial scale following approval.

If any serious adverse events occur, clinical trials or commercial distribution of any product candidates or products we develop could be suspended or terminated, and our business could be seriously harmed. Treatment-related side effects could also affect patient recruitment and the ability of enrolled patients to complete the trial or result in potential liability claims. Regulatory authorities could order us to cease further development of, deny approval of, and/or require us to cease selling our products or product candidates, if approved, for any or all targeted indications. If we are required to delay, suspend or terminate any clinical trial or commercialization efforts, the commercial prospects of our products or product candidates may be harmed, and our ability to generate product revenues from them or other product candidates that we develop may be delayed or eliminated. Additionally, if one or more of our product candidates receives marketing approval and we or others later identify undesirable side effects or adverse events caused by such products, a number of potentially significant negative consequences could result, including but not limited to:

- regulatory authorities may suspend, limit or withdraw approvals of such product, or seek an injunction against its manufacture or distribution;
- regulatory authorities may require additional warnings on the label, including “boxed” warnings, or issue safety alerts, Dear Healthcare Provider letters, press releases or other communications containing warnings or other safety information about the product;
- we may be required to change the way the product is administered or conduct additional clinical trials or post-approval studies;
- we may be required to create a risk evaluation and mitigation strategy, or REMS, which could include a medication guide outlining the risks of such side effects for distribution to patients;
- we may be subject to fines, injunctions or the imposition of criminal penalties;
- we could be sued and held liable for harm caused to patients; and
- our reputation may suffer.

Any of these events could prevent us from achieving or maintaining market acceptance of our products or any of our product candidates, if approved, and could seriously harm our business.

We may expend our limited resources to pursue a particular product candidate or indication and fail to capitalize on product candidates or indications that may be more profitable or for which there is a greater likelihood of success.

Because we have limited financial and management resources, we focus on research programs and product candidates that we identify for specific indications. As a result, we may forego or delay pursuit of opportunities with other product candidates or for other indications that later prove to have greater commercial potential. Our resource allocation decisions may cause us to fail to capitalize on viable commercial drugs or profitable market opportunities. Our spending on current and future research and development programs and product candidates for specific indications may not yield any commercially viable products. If we do not accurately evaluate the commercial potential or target market for a particular product candidate, we may relinquish valuable rights to that product candidate through collaboration, licensing or other royalty arrangements in cases in which it would have been more advantageous for us to retain sole development and commercialization rights to such product candidate.

Social issues around the abuse of opioids and stimulants, including law enforcement concerns over diversion and regulatory efforts to combat abuse, could decrease the potential market for our approved products or any of our other applicable product candidates.

Media stories regarding prescription drug abuse and the diversion of opioids, stimulants and other controlled substances are commonplace. Law enforcement and regulatory agencies may apply policies that seek to limit the availability of opioids and stimulants. Such efforts may inhibit the ability to commercialize our approved products or any of our other applicable product candidates. Aggressive enforcement and unfavorable publicity regarding, for example, the use or misuse of hydrocodone or other opioid drugs and stimulants, the limitations of abuse-deterrent formulations, public inquiries and investigations into prescription drug abuse, litigation or regulatory activity, sales, marketing, distribution or storage of our products could harm our reputation. Such negative publicity could reduce the potential size of the market for our approved products or any of our other applicable product candidates and decrease the revenue we are able to generate from their sale, if approved. Similarly, to the extent prescription drug abuse becomes a less prevalent or less urgent public health issue, regulators and third-party payors may not be willing to pay a premium for formulations with improved attributes of opioids or stimulants.

Additionally, efforts by the FDA and other regulatory bodies to combat abuse of opioids and stimulants may negatively impact the market for our approved products and any of our other applicable product candidates. For example, in April 2014, the FDA approved class-wide labeling changes to the indications for use of all approved ER/LA opioids, so that ER/LA opioids will be indicated only for the management of pain severe enough to require daily, around-the-clock, long-term opioid treatment and for which alternative treatment options are inadequate. These changes have reduced the number of prescriptions for opioids written by physicians and negatively impact the potential market our applicable product candidates. The FDA also held a public meeting in October 2014, on the development and regulation of abuse-deterrent formulations of opioid medications. Further, the Centers for Disease Control and Prevention previously issued draft guidelines for the prescribing of opioids for chronic pain, providing recommendations for primary care providers prescribing opioids for chronic pain on when to initiate or continue opioids, opioid selection and discontinuation, and the assessment of the risk and addressing harms of opioid use, among other areas. It is possible that FDA, or other regulatory bodies, will announce new regulatory initiatives at any time that may increase the regulatory burden or decrease the commercial opportunity for our approved products or any of our other applicable product candidates.

If the Arimoclomol EAP is terminated prior to commercialization of arimoclomol, if approved, it will have a material adverse effect on our business, results of operations, cash flows, financial condition and/or prospects.

Arimoclomol is currently available to NPC patients in the United States France, Germany, and other EU member states through the Arimoclomol EAP. The EAP is expected to remain in place until arimoclomol becomes commercially available in each of the current EAP markets. If the Arimoclomol EAP is terminated prior to commercialization of arimoclomol, it will have a material adverse effect on our business, results of operations, cash flows, financial condition and/or prospects.

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Risks Related to Our Business, Our Financial Position and Our Capital Needs

We may be unable to integrate the Acer business successfully or realize the anticipated synergies and related benefits of the Merger.

We entered into the Merger Agreement with Acer with the expectation that the Merger will result in various benefits and synergies. However, the Merger involved the combination of two previously independent public companies. In particular, prior to the signing of the Merger Agreement and the extension of the Bridge Loan, Acer's assets consisted primarily of approximately $0.6 million in cash or cash equivalents and certain product rights. Moreover, prior to the Merger Agreement, Acer had historically been unable to fund its operations on a standalone basis without substantial additional investment. We may be unable to successfully operate Acer's business or integrate it into our own operations as a combined company.

We are required to devote significant management attention and resources to integrating the portfolio and operations of Acer. Potential difficulties that we may encounter in the integration process include the following:

- the inability to combine our business with Acer's in a manner that permits us to achieve the cost savings or other synergies anticipated as a result of the Merger or to achieve such cost savings or other anticipated synergies in a timely manner, which could result in Zevra not realizing some anticipated benefits of the Merger in the time frame currently anticipated, or at all;

- the inability to realize the anticipated value from various Acer assets;

- the inability to coordinate and integrate research and development teams across technologies and products to enhance product development;

- the inability to integrate and manage personnel from the companies and minimizing the loss of key employees;

- the inability to consolidate our administrative and information technology infrastructure and financial systems and identify and eliminate redundant and underperforming functions and assets;

- the inability to harmonize our operating practices, employee development and compensation programs, internal controls and other policies, procedures and processes;

- the inability to coordinate distribution and marketing efforts;

- potential unknown liabilities and unforeseen increased expenses, delays or unfavorable conditions in connection with the post-closing integration; and

- performance shortfalls as a result of the diversion of management's attention from ongoing business activities as a result of the Merger and integrating our operations.

It is possible that the integration process could result in the distraction of our management, the loss of key employees, the disruption of our ongoing business or inconsistencies in our operations, services, standards, controls, procedures and policies, any of which could adversely affect our ability to maintain relationships with third parties and employees or to achieve the anticipated benefits of the Merger, or could otherwise adversely affect our business and financial results.

Our future results will suffer if we do not effectively manage our expanded operations following the Merger.

Following the Merger, the size and scope of our operations increased. We may continue to expand our size and operations through additional acquisitions or other strategic transactions. Our future success depends, in part, upon our ability to manage our expanded business, which may pose substantial challenges for our management, including challenges related to the management and monitoring of new operations and locations and associated increased costs and complexity. There can be no assurances that we will be successful in managing our expanded business or that we will realize the expected economies of scale, synergies and other benefits currently anticipated from the Merger or anticipated from any additional acquisitions or strategic transactions.

We may not be able to retain suppliers or distributors, or suppliers or distributors may seek to modify contractual relationships with us, which could have an adverse effect on our business and operations. Third parties may terminate or alter existing contracts or relationships with us.

We may experience impacts on relationships with customers, suppliers and distributors that may harm our business and results of operations. Certain suppliers or distributors may seek to terminate or modify contractual obligations following the Merger whether or not contractual rights are triggered as a result of the Merger. There can be no guarantee that our or Acer's prior customers, suppliers and distributors will remain with or continue to have a relationship with us or do so on contractual terms amenable to us. If any suppliers or distributors seek to terminate or modify contractual obligations or discontinue their relationship with us, then our business and results of operations may be harmed.

The restatement of our consolidated financial statements has subjected us to a number of additional risks and uncertainties, including increased possibility of legal proceedings. Any failure to maintain effective internal control over financial reporting could harm us.

As discussed elsewhere in this Annual Report, on March 25, 2024, the Audit Committee, after discussion with senior management and the Company's independent registered public accountants concluded that the Company's Prior Financial Statements and Prior Interim Financial Statements should no longer be relied upon. We have restated our consolidated financial statements for the year ended December 31, 2022, and the Prior Interim Financial Statements in this Annual Report on Form 10-K. The restatement of our consolidated financial statements has caused us to incu substantial expenses for legal, accounting, and other professional services and has diverted our management's attention from our business and could continue to do so. In addition, as a result o the restatement, investors may lose confidence in our financial reporting, the price of our common stock could decline and we may be subject to litigation or regulatory enforcement actions.

Management recently identified a material weakness in our internal control over financial reporting, which could have a significant adverse effect on our business and the price of our common stock.

Our management is required to report annually on the effectiveness of our internal control over financial reporting pursuant to Section 404 of the Sarbanes-Oxley Act, or Section 404. The rules governing the standards that must be met for our management to assess our internal control over financial reporting are complex and require significant documentation, testing and possible remediation.

A material weakness is a deficiency, or a combination of deficiencies, in internal control over financial reporting, such that there is a reasonable possibility that a material misstatement of the Company's annual or interim financial statements will not be prevented or detected on a timely basis. We have identified a material weakness in our internal controls relating to the accounting fo certain common stock warrants as liabilities, which led to understatements of derivative and warrant liability and additional paid-in capital and fluctuations in fair value adjustment related t derivative and warrant liability during the impacted periods. As a result, management has concluded that our internal control over financial reporting and our disclosure controls and procedure were, in each case as of December 31, 2023, ineffective. As described in Part II, Item 9A of this Annual Report on Form 10-K, management is taking steps to remediate the material weakness There can be no assurance that any measures we take will remediate the material weakness identified, nor can there be any assurance as to how quickly we will be able to remediate this materia weakness.

Our failure to certify the effectiveness of our internal control over financial reporting or our disclosure controls and procedures, or the identification of the material weakness, could subject us t regulatory scrutiny and a loss of public confidence, which could have a material adverse effect on our business and our stock price. In the future, we may identify additional material weaknesses or significant deficiencies, and we may not be able to remediate them in time to meet the deadline imposed by the Sarbanes-Oxley Act for compliance with the requirements of Section 404. In addition, we may encounter problems or delays in completing the implementation of any requested improvements and, if and when such a report is required, receiving a favorable attestation report from our independent registered public accounting firm. If we do not maintain adequate financial and management personnel, processes and controls, we may not be able to manage ou business effectively or accurately report our financial performance on a timely basis, which could cause a decline in our common stock price and adversely affect our results of operations and financial condition.

We have incurred significant recurring negative net operating cash flows. We expect to incur minimal positive net operating cash flows or negative net operating cash flows over the next several years and may never achieve or maintain profitability.

We have historically had significant negative net operating cash flows since inception. For the years ended December 31, 2023, and 2022, net cash used in operations was $33.5 million and $18.7 million, respectively. We have financed our operations through December 31, 2023, with funds raised in private placements of redeemable convertible preferred stock, the issuance of convertible promissory notes and term debt, our initial public offering and other public and private offerings of our common stock, as well as through revenue received under the AZSTARYS License Agreement, sales of arimoclomol under the Arimoclomol EAP, the Corium Consulting Agreement and, since the consummation of the Merger, sales of OLPRUVA.

Our recurring operating losses and negative cash flows from operations raise substantial doubt about our ability to continue as a going concern. We have no current source of revenues to sustain our present activities, and we do not expect to generate revenues until, and unless, the FDA or other regulatory agencies approve our product candidates, and we successfully commercialize our approved products and product candidates. Accordingly, our ability to continue as a going concern will require us to obtain additional financing to fund our operations. The perception of our inability to continue as a going concern may make it more difficult for us to obtain financing for the continuation of our operations and could result in the loss of confidence by investors, suppliers and employees. We have devoted substantially all of our financial resources and efforts to research and development, including preclinical studies and clinical trials, and commercialization. We are still in the early stages of commercializing our approved products and in development of many of our product candidates. We have devoted substantially all of our financial resources and efforts to research and development, including preclinical studies and clinical trials. We are in various stages of development of our product candidates, and we have only completed development of, and received regulatory approval for AZSTARYS and OLPRUVA. We expect to continue to incur significant expenses and operating losses over the next several years and our net losses may fluctuate significantly from quarter to quarter and year to year as we:

- continue to integrate the operations of Acer following the recent Merger;
- continue our ongoing preclinical studies, clinical trials and our product development activities for our pipeline of product candidates;
- seek regulatory approvals for product candidates that successfully complete clinical trials;
- continue research and preclinical development and initiate clinical trials of our product candidates;
- seek to discover and develop additional product candidates either internally or in partnership with other pharmaceutical companies;
- adapt our regulatory compliance efforts to incorporate requirements applicable to marketed products;
- maintain, expand and protect our intellectual property portfolio;
- incur additional legal, accounting and other expenses in operating as a public company; and
- add operational systems and personnel, if needed, to support any future commercialization efforts.

To become and remain profitable, we must succeed in developing and eventually commercializing prodrugs that generate significant revenue. This will require us to be successful in a range of challenging activities, including completing preclinical studies and clinical trials and obtaining regulatory approval of our product candidates, and manufacturing, marketing and selling, whether ourselves or through a license with a third party, any of our product candidates for which we may obtain regulatory approval, as well as discovering and developing additional product candidates. We are in various stages of these activities for our product candidates and we cannot guarantee that any strategy we adopt will be successful. We may never succeed in commercialization activities and, even if we do, may never generate revenue that is significant enough to achieve profitability.

Because of the numerous risks and uncertainties associated with prodrug development, we are unable to accurately predict the timing or amount of expenses or when, or if, we will be able to achieve profitability. If we are required by regulatory authorities to perform studies in addition to those currently expected, or if there are any delays in the initiation and completion of our clinical trials or the development of any of our product candidates, our expenses could increase.

Even if we achieve profitability, we may not be able to sustain or increase profitability on a quarterly or annual basis. Our failure to become and remain profitable would depress our value and could impair our ability to raise capital, expand our business, maintain our research and development efforts, obtain product approvals, diversify our product offerings or continue our operations. A decline in our value could also cause you to lose all or part of your investment.

Identifying potential product candidates and conducting preclinical studies and clinical trials is a time-consuming, expensive and uncertain process that takes years to complete, and we may not generate the necessary data or results required to obtain regulatory approval for our product candidates or claims necessary to make such candidates profitable and achieve product sales. In addition, our approved products or any of our product candidates, if approved, may not achieve commercial success. Our commercial revenue will be derived from sales of prodrug products. We cannot guarantee that we or Corium will be able to successfully commercialize OLPRUVA or AZSTARYS to any certain level, or any of the product candidates subject to the AZSTARYS License Agreement, even if approved, or that we will ever receive any additional payments under the AZSTARYS License Agreement from the commercial sales of AZSTARYS or any future payments under the AZSTARYS License Agreement. Accordingly, we will need to continue to rely on additional financing to achieve our business objectives. Adequate additional financing may not be available to us on acceptable terms, or at all. To the extent that we raise additional capital through the sale of equity or debt securities, the terms of those securities or debt may restrict our ability to operate. Any future debt financing and equity financing, if available, may involve agreements that include, covenants limiting and restricting our ability to take specific actions, such as incurring additional debt, making capital expenditures, entering into profit-sharing or other arrangements or declaring dividends. If we raise additional funds through collaborations, strategic alliances or marketing, distribution or licensing arrangements with third parties, we may be required to relinquish valuable rights to our technologies, future revenue streams, research programs or product candidates or to grant licenses on terms that may not be favorable to us. In addition, we may seek additional capital due to favorable market conditions or strategic considerations even if we believe we have sufficient funds for our current or future operating plans. If we are unable to raise capital when needed or on attractive terms, we could be forced to delay, reduce or altogether cease our research and development programs or future commercialization efforts.

The auditor's opinion on our audited financial statements for the fiscal year ended December 31, 2023, included in this annual report on Form 10-K, contains an explanatory paragraph relating to our ability to continue as a going concern.

The auditor's opinion on our audited financial statements for the year ended December 31, 2023, includes an explanatory paragraph stating that our recurring losses from operations, stockholders' deficit and negative operating cash flows raise substantial doubt about our ability to continue as a going concern. While we believe that we will be able to raise the capital we need to continue our operations, there can be no assurances that we will be successful in these efforts or will be able to resolve our liquidity issues or eliminate our operating losses. If we are unable to obtain sufficient funding, we would need to significantly reduce our operating plans and curtail some or all of our product development, commercialization, and strategic plans. Accordingly, our business, prospects, financial condition and results of operations will be materially and adversely affected, and we may be unable to continue as a going concern. If we are unable to continue as a going concern, we may have to liquidate our assets and may receive less than the value at which those assets are carried on our audited consolidated financial statements, and it is likely that investors will lose all or a part of their investment. If we seek additional financing to fund our business activities in the future and there remains substantial doubt about our ability to continue as a going concern, investors or other financing sources may be unwilling to provide additional funding on commercially reasonable terms or at all.

We may need additional funding to pursue our business objectives. If we are unable to raise capital when needed, we could be forced to delay, reduce or altogether cease our prodrug development programs or commercialization efforts or cease operations altogether.

Based on our current operating plan, our existing resources are expected to be sufficient to fund operating expense and capital investment requirements into, but not through, 2026. However, unless we are able to restructure the amounts outstanding on our margin loan facility, we may be required to repay the loan and thereby deplete the cash available to fund our operations. Because of this, the auditor's opinion on our audited financial statements for the year ended December 31, 2023, includes an explanatory paragraph stating that our recurring losses from operations and negative cash flows raise substantial doubt about our ability to continue as a going concern. We do not currently have sufficient funds to finance our continuing operations beyond the short-term or to further advance any or our product candidates further into clinical development and commercialization. In order to continue to commercialize our approved products and advance development of our product candidates, we will need to obtain substantial additional funding in connection with our continuing operations from one or more equity offerings, debt financings or other third-party funding, including potential strategic alliances and licensing or collaboration arrangements, and we cannot guarantee that we will be able to generate sufficient proceeds from sales, or be successful in completing other transactions, that will fund our operating expenses. If we are delayed in obtaining additional funding or are unable to complete a strategic transaction, we may discontinue our development activities on our product candidates or discontinue our operations. Even if we are able to fund continued development and any of our other product candidates is approved, we expect that we will need to complete a strategic transaction or raise substantial additional funding through public or private debt or equity securities to successfully commercialize any approved product or other product candidate.

We expect that our only sources of revenues will be through payments arising from sales of our approved products or potential consulting arrangements and any other future arrangements related to one of our product candidates. We cannot guarantee that we will be able to generate sufficient proceeds from the sale of our approved products or product candidates, if approved, or be successful in completing other transactions that will fully fund our operating expenses. Further, the recent economic uncertainty, may dramatically reduce our ability to secure debt or equity financing necessary to support our operations. If we are delayed in obtaining additional funding or are unable to complete a strategic transaction, we may discontinue our development activities on our product candidates or discontinue our operations. Our future capital requirements will depend on many factors, including:

- the progress and results of our preclinical studies, clinical trials, chemistry, manufacturing and controls (“CMC ”), and other product development and commercialization activities;
- the scope, progress, results and costs of preclinical development, laboratory testing and clinical trials for our product candidates;
- the ability to obtain differentiating claims in the labels for our product candidates;
- the number and development requirements of other product candidates that we may pursue;
- the costs, timing and outcome of regulatory review of our product candidates;
- the efforts necessary to institute post-approval regulatory compliance requirements;
- the costs and timing of future commercialization activities, including product manufacturing, marketing, sales and distribution, for any of our product candidates for which we receive marketing approval;
- the commercial revenue received from commercial sales of our approved products, or any of our product candidates subject to the terms of the AZSTARYS License Agreement, or sales of our product candidates for which we receive marketing approval in the future, which may be affected by market conditions, including obtaining coverage and adequate reimbursement of our approved products, or any of our product candidates, from third-party payors, including government programs and managed care organizations, and competition within the therapeutic class to which our approved products, or any our product candidates are assigned;
- the success in commercializing our approved products;
- the costs and timing of preparing, filing and prosecuting patent applications, maintaining and enforcing our intellectual property rights and defending any intellectual property-related claims; and
- the extent to which we acquire or in-license other product candidates and technologies.

Risks Related to Our Dependence on Third Parties

We rely on and expect to continue to rely on third parties to conduct our clinical trials for our product candidates, and those third parties may not perform satisfactorily, including failing to meet deadlines for the completion of such trials.

We have engaged and expect to continue to engage CROs for our planned clinical trials of our product candidates. We rely on and expect to continue to rely on CROs, as well as other third parties, such as clinical data management organizations, medical institutions and clinical investigators, to conduct those clinical trials. Agreements with such third parties might terminate for a variety of reasons, including a failure to perform by the third parties. If we need to enter into alternative arrangements, our drug development activities would be delayed.

Our reliance on these third parties for research and development activities reduces our control over these activities but does not relieve us of our responsibilities. For example, we remain responsible for ensuring that each of our clinical trials is conducted in accordance with the general investigational plan and protocols for the trial. Moreover, the FDA requires us to comply with regulatory standards, commonly referred to as good clinical practices, or GCPs, for conducting, recording and reporting the results of clinical trials to assure that data and reported results are credible and accurate and that the rights, integrity and confidentiality of trial participants are protected. Regulatory authorities enforce these GCPs through periodic inspections of trial sponsors, investigators and trial sites. We also are required to register specified ongoing clinical trials and post the results of completed clinical trials on a government-sponsored database, ClinicalTrials.gov, within specified timeframes. In addition, we must conduct our clinical trials with product produced under cGMP requirements. Similar requirements apply in foreign jurisdictions. Failure to comply with these regulations may require us to repeat preclinical studies and clinical trials, which would delay the regulatory approval process. Failure to comply with the applicable requirements related to clinical investigations by us, our CROs or clinical trial sites can also result in clinical holds and termination of clinical trials, debarment, FDA or foreign regulatory authorities refusal to approve applications based on the clinical data, warning letters, withdrawal of marketing approval if the product has already been approved, fines and other monetary penalties, delays, adverse publicity and civil and criminal sanctions, among other consequences.

Furthermore, these third parties may also have relationships with other entities, some of which may be our competitors. If these third parties do not successfully carry out their contractual duties, meet expected deadlines or conduct our clinical trials in accordance with regulatory requirements or our stated protocols, we will not be able to obtain, or may be delayed in obtaining, marketing approvals for our product candidates and will not be able to, or may be delayed in our efforts to, successfully commercialize our product candidates.

In addition, investigators for our clinical trials may serve as scientific advisors or consultants to us from time to time and may receive cash or equity compensation in connection with such services. If these relationships and any related compensation result in perceived or actual conflicts of interest, or the FDA or foreign regulatory authorities conclude that the financial relationship may have affected the interpretation of the study, the integrity of the data generated at the applicable clinical trial site may be questioned and the utility of the clinical trial itself may be jeopardized, which could result in the delay or rejection of any NDA or foreign marketing authorization application we submit by the FDA or foreign regulatory authorities. Any such delay or rejection could prevent us from commercializing our product candidates. Further, our arrangements with investigators are also subject to scrutiny under other health care regulatory laws, such as the Anti-Kickback Statute and comparable foreign laws.

We also rely on and expect to continue to rely on other third parties to store and distribute product supplies for our clinical trials. Any performance failure on the part of our distributors could delay clinical development or marketing approval of our product candidates or commercialization of our products, producing additional losses and depriving us of potential product revenue.

If the third parties with whom we contract do not successfully carry out their contractual duties or obligations or meet expected deadlines or if the quality or accuracy of the clinical data they obtain is compromised due to the failure to adhere to our clinical protocols or regulatory requirements or for other reasons, our clinical trials may be extended, delayed or terminated, we may need to conduct additional trials, and we may not be able to obtain regulatory approval for or successfully commercialize our product candidates. As a result, the commercial prospects for our product candidates would be harmed, our costs could increase and our ability to generate revenue could be delayed. To the extent we are unable to successfully identify and manage the performance of third-party service providers in the future, our business may be adversely affected.

We contract with third parties for the manufacture of our approved products, partnered products and product candidates that utilize SDX, sodium phenylbutyrate, and arimoclomol, and we expec to continue to do so. This reliance on third-party manufacturers increases the risk that we will not have sufficient quantities of SDX, sodium phenylbutyrate and arimoclomol, or such quantities a an acceptable cost, which could delay, prevent or impair commercialization or development efforts.

We do not have any manufacturing facilities. We procure the bulk drug substances for our approved products and product candidates from sole-source, third-party manufacturers and the partnered products and product candidates that utilize these moieties as the API used in our clinical trials from other third parties. We anticipate we will continue to do so for the foreseeable future. We also expect to continue to rely on third parties as we proceed with preclinical and clinical testing of our product candidates, as well as for commercial manufacture of our approved products, or any of our product candidates should such candidates receive marketing approval. This reliance on third parties increases the risk that we will not have sufficient quantities of SDX, sodium phenylbutyrate, arimoclomol, or other bulk drug substances or our approved products, partnered product or product candidates, or such quantities at an acceptable cost or quality, which could delay, prevent or impair our ability to timely conduct our clinical trials or our other development or commercialization efforts.

We may be unable to establish any future agreements with third-party manufacturers or to do so on acceptable terms. Even if we are able to maintain our existing third-party relationships or establish any such agreements with other third-party manufacturers, reliance on third-party manufacturers entails additional risks, including:

- reliance on the third party for FDA and DEA and comparable foreign authorities regulatory compliance and quality assurance;
- the possible misappropriation of our proprietary information, including our trade secrets and know-how;
- disruption and costs associated with changing suppliers, including additional regulatory filings;
- the possible breach, termination or nonrenewal of the agreement by the third party at a time that is costly or inconvenient for us;
- a delay or inability to procure or expand sufficient manufacturing capacity;
- manufacturing and product quality issues related to scale-up of manufacturing;
- costs and validation of new equipment and facilities required for scale-up;
- the inability to negotiate manufacturing agreements with third parties under commercially reasonable terms;
- termination or nonrenewal of manufacturing agreements with third parties in a manner or at a time that is costly or damaging to us;
- the reliance on a limited number of sources, and in some cases, single sources for product components, such that if we are unable to secure a sufficient supply of these product components, we will be unable to manufacture and sell our product candidates in a timely fashion, in sufficient quantities or under acceptable terms; and
- carrier disruptions or increased costs that are beyond our control.

Any of these events could lead to clinical trial delays, failure to obtain regulatory approval or impact our ability to successfully commercialize our products, if approved. Some of these events could be the basis for FDA or foreign regulatory authorities action, including injunction, recall, seizure or total or partial suspension of production.

The facilities used by our contract manufacturers to manufacture our approved products, and any of our product candidates are subject to review by the FDA pursuant to inspections that will be conducted after we submit our marketing application to the FDA, and such inspections could result in findings that lead to failure to obtain FDA approval of such marketing applications.

We do not, other than through our contractual arrangements, control the manufacturing process of our approved products, or any of our other product candidates, and we are completely dependent on, our contract manufacturing partners for compliance with cGMP requirements and for manufacture of both active drug substances and finished drug products. If our contract manufacturers cannot successfully manufacture material that conforms to our specifications and the strict regulatory requirements of the FDA or other regulatory authorities, we will not be able to secure and maintain regulatory approval of our marketing applications for the use of their manufacturing facilities for our products. In addition, other than through our contractual agreements, we have no control over the ability of our contract manufacturers to maintain adequate quality control, quality assurance and qualified personnel. If the FDA, or a comparable foreign regulatory authority, does not approve these facilities for the manufacturing of our approved products, or any of our other product candidates, or if it withdraws any such approval in the future, we may need to find alternative manufacturing facilities, which would significantly impact our ability to develop, obtain marketing approval for or market our approved products, or any of our other product candidates, if approved.

Further, for our approved products and any of our product candidates, if approved, our suppliers will be subject to regulatory requirements, covering manufacturing, testing, quality control and record keeping relating to our approved products or any of our product candidates, if approved, and subject to ongoing inspections by the regulatory agencies. Failure by any of our suppliers to comply with applicable regulations may result in long delays and interruptions to our manufacturing capacity while we seek to secure another supplier that meets all regulatory requirements, as well as market disruption related to any necessary recalls or other corrective actions.

Third-party manufacturers may not be able to comply with cGMP regulations or similar regulatory requirements outside the United States. Our failure, or the failure of our third-party manufacturers, to comply with applicable regulations could result in sanctions being imposed on us, including warning letters, clinical holds or termination of clinical trials, fines, injunctions, restitution, disgorgement, civil penalties, delays, suspension or withdrawal of approvals or other permits, FDA refusal to approve pending applications, product detentions, FDA or DEA consent decrees placing significant restrictions on or suspending manufacturing and distribution operations, debarment, refusal to allow import or export, product detentions, adverse publicity, dear-health-care-provider letters or other warnings, license revocation, seizures or recalls of product candidates, operating restrictions, refusal of government contracts or future orders under existing contracts and civil and criminal liability, including False Claims Act liability, exclusion from participation in federal health care programs, and corporate integrity agreements among other consequences, any of which could significantly and adversely affect supplies of our prodrugs.

Our product candidates and any prodrugs that we may develop may compete with other product candidates and drugs for access to manufacturing facilities, and we may be unable to obtain access to these facilities on favorable terms.

There are a limited number of manufacturers that operate under cGMP or comparable foreign regulations and that might be capable of manufacturing for us. Any performance failure on the part of our existing or future manufacturers could delay clinical development or marketing approval. We do not currently have arrangements in place for redundant supply or a second source for our approved products or product candidates bulk drug substance. If our current contract manufacturer for our approved products or product candidates bulk drug substance cannot perform as agreed, we may be required to replace such manufacturer and we may incur added costs and delays in identifying and qualifying any such replacement. Any performance failure or significant delay could negatively affect our business, results of operations and financial condition.

We have entered into collaborations with Commave, to develop, manufacture and commercialize AZSTARYS worldwide. In addition, we may seek collaborations with third parties for the development or commercialization of our other product candidates, or in other territories. If those collaborations are not successful, we may not be able to capitalize on the market potential of AZSTARYS or any of our other product candidates, if approved.

We entered into the AZSTARYS License Agreement with Commave pursuant to which we granted an exclusive, worldwide license to Commave to develop, manufacture and commercialize AZSTARYS. Corium was tasked by Commave to lead all commercialization activities for AZSTARYS under the AZSTARYS License Agreement. We cannot guarantee that the AZSTARYS License Agreement will be successful or that we will receive any future payments under the AZSTARYS License Agreement. For instance, Commave has the option to terminate the AZSTARYS License Agreement, in its entirety or on a product-by-product and country-by-country basis, at their convenience either (i) prior to the first regulatory approval of a product upon sixty days prior written notice or (ii) subsequent to the first regulatory approval of a product upon one hundred twenty days prior written notice. Further, even if Commave does not terminate the AZSTARYS License Agreement, we cannot guarantee that we will receive any additional milestone or royalty payments under the AZSTARYS License Agreement. In addition, under the AZSTARYS License Agreement, we have limited control over the amount and timing of resources that Corium will dedicate to the development, manufacturing or commercialization of AZSTARYS, and we may not always agree with Corium's efforts. Our ability to generate revenue under the AZSTARYS License Agreement will depend, in part, on Corium's ability to successfully perform the functions assigned to it under the AZSTARYS License Agreement.

We may also seek additional third-party collaborators for the development or commercialization of any of our other product candidates, which are not subject to the AZSTARYS License Agreement, or those that are subject to the AZSTARYS License Agreement, but the option is not exercised by Commave. In such cases, our likely collaborators would include large and mid-size pharmaceutical companies, regional, national and international pharmaceutical companies and biotechnology companies. If we do enter into any such collaboration arrangements with any third parties, we will likely have limited control over the amount and timing of resources that our collaborators dedicate to the development or commercialization of our approved products or any of our other product candidates. Our ability to generate revenue from these arrangements will depend on our collaborators' abilities to successfully perform the functions assigned to them in these arrangements.

Our collaboration with Commave, or Collaborator, pose the following risks to us:

- the Collaborator has significant discretion in determining the efforts and resources that they will apply to these collaborations;
- the Collaborator may not perform their obligations as expected;
- the Collaborator may not pursue commercialization of AZSTARYS, any of our product candidates covered under the AZSTARYS License Agreement, if approved, or may elect not to continue or renew commercialization programs based on post-approval clinical trial results, changes in a Collaborator's strategic focus or available funding, or external factors, such as an acquisition, that divert resources or create competing priorities;
- the Collaborator could independently develop, or develop with third parties, products that compete directly or indirectly with AZSTARYS, or any of our other products covered under the AZSTARYS License Agreement, as applicable, if the Collaborator believes that competitive products are more likely to be successfully developed or can be commercialized under terms that are more economically attractive than ours;
- AZSTARYS, any of our other products covered under the AZSTARYS License Agreement, if approved, may be viewed by the Collaborator as competitive with their own product candidates or products, which may cause the Collaborator to cease to devote resources to the commercialization of AZSTARYS, or any of our other products covered under the AZSTARYS License Agreement, if approved;
- the Collaborator may not commit sufficient resources to the development, marketing and distribution of AZSTARYS and any of our other products covered under the AZSTARYS License Agreement, as applicable;
- disagreements with the Collaborator, including disagreements over proprietary rights, contract interpretation or the preferred course of development or commercialization, might cause delays or termination of the development or commercialization of AZSTARYS, or any of our other products covered under the AZSTARYS License Agreement, as applicable, might lead to additional responsibilities for us with respect to AZSTARYS or any of our other products covered under the AZSTARYS License Agreement, or might result in litigation or arbitration, any of which would be time-consuming and expensive;
- the Collaborator may not properly maintain or defend our or their intellectual property rights or may use our or their proprietary information in such a way as to invite litigation that could jeopardize or invalidate such intellectual property or proprietary information or expose us to potential litigation;
- the Collaborator may infringe the intellectual property rights of third parties, which may expose us to litigation and potential liability; and
- the license agreements may be terminated by the Collaborator under specified circumstances and, if terminated, we could be required to raise additional capital to pursue further development or commercialization of AZSTARYS or any of our other products covered under the AZSTARYS License Agreement.

If we enter into any future collaborations we will face similar risks with any future collaborators as well.

The AZSTARYS License Agreement and any other licensing or collaboration agreements we may enter into may not lead to commercialization of AZSTARYS, or development of KP1077, or any of our other product candidates in the most efficient manner or at all. If Corium or a future collaborator of ours were to be involved in a business combination, the continued pursuit and emphasis on our drug development or commercialization program could be delayed, diminished or terminated.

If we are not able to establish collaborations for our product candidates, we may have to alter our development and commercialization plans.

Our prodrug development programs and the potential commercialization of our product candidates, if approved, will require substantial additional capital. For our product candidates, which are not subject to the terms of the AZSTARYS License Agreement, we may need to collaborate with pharmaceutical and biotechnology companies for the development and potential commercialization of those product candidates.

We face significant competition in seeking appropriate collaborators. Whether we reach a definitive agreement for a collaboration will depend, among other things, upon our assessment of the collaborator's resources and expertise, the terms and conditions of the proposed collaboration and the proposed collaborator's evaluation of a number of factors. Those factors may include the design or results of clinical trials, the likelihood of approval by the FDA or similar regulatory authorities outside the United States, the potential market for the subject product candidate, the costs and complexities of manufacturing and delivering such product candidate to patients, the potential of competing products, the existence of uncertainty with respect to our ownership of technology, which can exist if there is a challenge to such ownership without regard to the merits of the challenge, and industry and market conditions generally.

The collaborator may also consider alternative product candidates or technologies for similar indications that may be available to collaborate on and whether such a collaboration could be more attractive than the one with us for our product candidate.

Collaborations are complex and time-consuming to negotiate and document. In addition, there have been a significant number of recent business combinations among large pharmaceutical companies that have resulted in a reduced number of potential future collaborators.

We may not be able to negotiate collaborations on a timely basis, on acceptable terms, or at all. If we are unable to do so, we may have to curtail the development of product candidates, reduce or delay one or more of our development programs, delay potential commercialization of our product candidates or reduce the scope of any sales or marketing activities of our product candidates, or increase our expenditures and undertake development or commercialization activities at our own expense of our product candidate. If we elect to increase our expenditures to fund development or commercialization activities on our own, we may need to obtain additional capital, which may not be available to us on acceptable terms or at all. If we do not have sufficient funds, we may not be able to further develop our product candidates or bring our product candidates to market and generate product revenue.

Provisions in our agreements with Aquestive and Commave may inhibit our ability to enter into future collaborations with third parties.

We are party to a termination agreement with Aquestive Therapeutics, or Aquestive, that may limit the value of any sale, license or commercialization of AZSTARYS or KP1077. Under this termination agreement, Aquestive has the right to receive a royalty amount equal to 10% of any value generated by AZSTARYS or KP1077, and any product candidates which contain SDX, including royalty payments on any license of AZSTARYS or KP1077, the sale of AZSTARYS or KP1077 to a third party or the commercialization of AZSTARYS or KP1077.

We also granted to Commave a right of first refusal to acquire, license or commercialize any additional product candidate which contains SDX and is intended to treat ADHD or any other CNS disorder with such right of first refusal expiring upon the acceptance of a new drug application for such product candidate. We also granted Commave a right of first negotiation and a right of first refusal, subject to specified exceptions, for any assignment of our rights under the AZSTARYS License Agreement. We cannot predict if these obligations will limit the value we may receive from any future sale or license of any additional product candidate.

Risks Related to Our Intellectual Property

If we are unable to obtain and maintain trade secret protection or patent protection for our technology, our approved products or our product candidates, or if the scope of the patent protection obtained is not sufficiently broad, our competitors could develop and commercialize technology and drugs similar or identical to ours, and our ability to successfully commercialize our technology, our approved products, or our product candidates, if approved, may be impaired.

Our success depends in large part on our ability to obtain and maintain trade secret protection of our proprietary LAT platform technology as well as patent protection in the United States and other countries with respect to our approved products, and any of our product candidates. We seek to protect our proprietary position by filing patent applications in the United States and abroad related to our product technology and product candidates. As part of the AZSTARYS License Agreement, Commave obtained from us an exclusive, worldwide license to certain patents that cover AZSTARYS.

The patent prosecution process is expensive and time-consuming, and we may not be able to file and prosecute all necessary or desirable patent applications at a reasonable cost or in a timely manner. It is also possible that we will fail to identify patentable aspects of our research and development output before it is too late to obtain patent protection. We may not have the right to control the preparation, filing and prosecution of patent applications, or to maintain the rights to patents, licensed to third parties by us.

Further, we may also not have the right to control the preparation, filing and prosecution of patent applications, or to maintain the rights to patents, licensed from third parties to us. Therefore, any such patents and applications may not be prosecuted and enforced in a manner consistent with the best interests of our business. If such licensors or licensees fail to maintain such patents, or lose rights to those patents, the rights we have in- or out-licensed may be reduced or eliminated.

The patent position of biotechnology and pharmaceutical companies, generally, is highly uncertain, involves complex legal and factual questions and has in recent years been the subject of much litigation. In addition, the laws of foreign countries may not protect our rights to the same extent as the laws of the United States or visa-versa. For example, European patent law restricts the patentability of methods of treatment of the human body more than United States law. Publications of discoveries in the scientific literature often lag behind the actual discoveries, and utility, or equivalent, patent applications in the United States and other jurisdictions are typically, for example, not published until 18 months after the filing date of such patent applications, or in some cases not at all. Therefore, we cannot know with certainty whether we were the first to make and/or use the inventions claimed in our owned or licensed patents or pending patent applications, or that we were the first to file for patent protection of such inventions. As a result, the issuance, scope, priority, validity, enforceability and commercial value of our patent rights are highly uncertain. Our pending and future patent applications may not result in patents being issued that protect our product candidates, in whole or in part, or which effectively prevent others from commercializing competitive technologies and drugs. Changes in either the patent laws or interpretation of the patent laws in the United States and other countries may diminish the value of our patents or narrow the scope of our patent protection.

Our patent position is subject to numerous additional risks, including the following:

- we may fail to seek patent protection for inventions that are important to our success;
- our pending patent applications may not result in issued patents;
- we cannot be certain that we are the first to invent the inventions covered by pending patent applications or that we are the first to file such applications and, if we are not, we may be subject to priority disputes or lose rights;
- we may be required to disclaim part or all of the term of certain patents or all of the term of certain patent applications;
- we may not be able to acquire patent term extensions or supplemental certificates of certain patents, domestic or foreign, due to regulatory delays, among others, which may affect the term of enforceability of such patents over time;
- we may file patent applications but have claims restricted or we may not be able to supply sufficient data to support our claims and, as a result, may not obtain the original claims desired or we may receive restricted claims; alternatively, it is possible that we may not receive any patent protection from an application;
- even if our owned and licensed patent applications issue as patents, they may not issue in a form that will provide us with any meaningful protection, and may not be of sufficient scope or strength to provide us with any commercial advantage;
- our competitors may be able to design around our owned or licensed patents by developing similar or alternative technologies or drugs without infringing on our intellectual property rights;
- we could inadvertently abandon a patent or patent application, resulting in the loss of protection of intellectual property rights in a particular country, and we, our collaborators or our patent counsel may take action resulting in a patent or patent application becoming abandoned which may not be able to be reinstated or if reinstated, may suffer patent term adjustments or loss;
- the claims of our issued patents or patent applications when issued may not cover our product candidates;
- no assurance can be given that our patents would be declared by a court or tribunal, domestic or foreign, to be valid or enforceable or that a competitor's technology or product would be found by a court or tribunal, domestic or foreign, to infringe our patents and our patents or patent applications may be challenged by third parties in patent litigation, domestic or foreign, or in proceedings before the United States Patent and Trademark Office, or the USPTO, or its foreign counterparts, and may ultimately be declared invalid or unenforceable or narrowed in scope;
- there may be prior art of which we are not aware that may affect the validity or enforceability of a patent claim and there may be prior art of which we are aware, but which we do not believe affects the validity or enforceability of a claim, which may, nonetheless, ultimately be found to affect the validity or enforceability of a claim;
- third parties may develop products that have the same or similar effect as our products without infringing our patents;
- third parties may intentionally circumvent our patents by means of alternate designs or processes or file applications or be granted patents that would block or hurt our efforts;
- there may be dominating or intervening patents relevant to our product candidates of which we are not aware;
- obtaining regulatory approval for pharmaceutical products is a lengthy and complex process, and as a result, any patents covering our product candidates may expire before or shortly after such product candidates are approved and commercialized;
- the patent and patent enforcement laws of some foreign jurisdictions do not protect intellectual property rights to the same extent as laws in the United States, and many companies have encountered significant difficulties in protecting and defending such rights in foreign jurisdictions; and
- we may not develop additional proprietary technologies that are patentable.

Any of these factors could hurt our ability to gain full patent protection for our products. Registered trademarks and trademark applications in the United States and other countries are subject to similar risks as described above for patents and patent applications, in addition to the risks described below.

Further, a third party may misappropriate or reverse engineer our proprietary LAT platform technology, which could limit our ability to stop others from using or commercializing similar or identical technology and resultant product candidates, product technology or prodrugs, or limit the duration of the trade secret protection of our proprietary LAT platform technology.

Moreover, we may be subject to a third-party pre-issuance submission of prior art to the USPTO, or become involved in opposition, litigation, nullity, derivation, reexamination, inter partes review, post-grant review or interference proceedings challenging our patent rights or the patent rights of others. An adverse determination in any such submission, proceeding or litigation could reduce the scope of, or invalidate, our patent rights, allow third parties to commercialize our technology or drugs and compete directly with us, without payment to us or result in our inability to manufacture or commercialize drugs without infringing third-party patent rights. In addition, if the breadth or strength of protection provided by our patents and patent applications is threatened, it could dissuade companies from collaborating with us to seek patent protection or to license, develop or commercialize current or future product candidates.

In addition, the issuance of a patent is not conclusive as to its inventorship, ownership, scope, priority, validity or enforceability, and our owned and licensed patents may be challenged in the courts, patent offices and tribunals in the United States and abroad. Such challenges may result in loss of exclusivity or in patent claims being narrowed, invalidated or held unenforceable, in whole or in part, which could limit our ability to stop others from using or commercializing similar or identical technology and drugs, or limit the duration of the patent protection of our product technology, product candidates and prodrugs.

Changes in patent law in the United States and other jurisdictions could alter or diminish the value of patents in general, thereby impairing our ability to protect our products and technologies.

The standards that the USPTO and patent offices in other countries use to grant patents are not always applied predictably or uniformly and can change. Changes in either the patent laws or interpretation of the patent laws in the United States and other countries could increase the uncertainties and costs, and may diminish our ability to protect our inventions, obtain, maintain, and enforce our intellectual property rights and, more generally, could affect the value of our intellectual property or narrow the scope of our owned and licensed patents.

In addition, the patent positions of companies in the development and commercialization of pharmaceuticals are particularly uncertain. The United States Supreme Court has ruled on several patent cases in recent years, either narrowing the scope of patent protection available in certain circumstances or weakening the rights of patent owners in certain situations. Depending on future actions by the United States Congress, the United States courts, the USPTO and the relevant law-making bodies in other countries, the laws and regulations governing patents could change in unpredictable ways that would weaken our ability to obtain new patents or to enforce our existing patents and patents that we might obtain in the future. For example, in the 2013 case *Assoc. for Molecular Pathology v. Myriad Genetics, Inc.*, the United States Supreme Court held that certain claims to DNA molecules are not patentable. While we do not believe that any of the patents owned or licensed by us will be found invalid based on this decision, future decisions by the courts, the United States Congress or the USPTO may impact the value of our patents.

For another example, on September 16, 2011, the Leahy-Smith America Invents Act, or the Leahy-Smith Act, was signed into law. The Leahy-Smith Act includes a number of significant changes to U.S. patent law. These include provisions that affect the way patent applications are prosecuted in the United States, redefine prior art and may also affect patent litigation. The USPTO recently developed new regulations and procedures to govern administration of the Leahy-Smith Act, and many of the substantive changes to patent law associated with the Leahy-Smith Act, and in particular, the first-to-file provisions, only became effective on March 16, 2013.

The Leahy-Smith Act and its implementation could increase the uncertainties and costs surrounding the prosecution of our patent applications and the enforcement or defense of our issued patents. For instance, the Leahy-Smith Act established the *inter partes* review and post grant review procedures that has lowered the burden of proof for invalidity challenges to issued patents and limited the ability to amend patent claims in response to such challenges. In addition, patent reform legislation may pass in the future that could lead to additional uncertainties and increased costs surrounding the prosecution, enforcement and defense of our owned and licensed patents and/or patent applications.

We may become involved in lawsuits to protect or enforce our patents or other intellectual property, which could be expensive, time consuming and unsuccessful.

Competitors or other third parties may infringe our patents, trademarks, copyrights or other intellectual property. To counter infringement or unauthorized use, we may be required to file infringement claims, which can be expensive and time consuming and divert the time and attention of our management and scientific personnel. Our pending patent applications cannot be enforced against third parties practicing the technology claimed in such applications unless and until a patent issues from such applications. Any claims we assert against perceived infringers could provoke these parties to assert counterclaims against us alleging that we infringe their patents, in addition to counterclaims asserting that our patents are invalid or unenforceable, or both. In patent litigation in the United States, defendant counterclaims alleging invalidity and/or unenforceability are commonplace. Grounds for a validity challenge could be an alleged failure to meet any of several statutory requirements, including lack of novelty, obviousness, non-enablement or insufficient written description. Grounds for an unenforceability assertion could be an allegation that someone connected with prosecution of the patent withheld relevant information from the USPTO or made a misleading statement during prosecution. The outcome following legal assertions of invalidity and unenforceability is unpredictable. In any patent infringement proceeding, there is a risk that a court will decide that a patent of ours is invalid or unenforceable, in whole or in part, and that we do not have the right to stop the other party from using the invention at issue. There is also a risk that, even if the validity of such patents is upheld, the court will construe the patent's claims narrowly or decide that we do not have the right to stop the other party from using the invention at issue on the grounds that our patent claims do not cover the invention, or decide that the other party's use of our patented technology falls under the safe harbor to patent infringement under 35 U.S.C. §271(e)(1). An adverse outcome in a litigation or proceeding involving our patents could limit our ability to assert our patents against those parties or other competitors and may curtail or preclude our ability to exclude third parties from making and selling similar or competitive products and technologies. Any of these occurrences could adversely affect our competitive business position, business prospects and financial condition. Similarly, if we assert trademark infringement claims, a court may determine that the marks we have asserted are invalid or unenforceable, or that the party against whom we have asserted trademark infringement has superior rights to the marks in question. In this case, we could ultimately be forced to cease use of such trademarks.

Third parties may initiate legal proceedings alleging that we are infringing their intellectual property rights, the outcome of which would be uncertain and could significantly harm our business.

Our commercial success depends upon our ability, and the ability of any collaborators, to develop, manufacture, market and sell our product candidates and use our proprietary technologies without infringing the proprietary rights of third parties. There is considerable intellectual property litigation in the biotechnology and pharmaceutical industries. In particular, we are focused on developing product candidates based on widely used therapeutic agents or drugs, many of which may be protected by proprietary rights of third parties.

Although we seek to develop proprietary prodrug formulations that do not infringe the intellectual property rights of others, we may become party to, or threatened with, future adversarial proceedings or litigation, domestic or foreign, regarding intellectual property rights with respect to our prodrugs or other aspects of our technology, including, for example, interference or derivation proceedings before the USPTO. Third parties may assert infringement claims against us based on existing patents or patents that may be granted in the future.

If we are found to infringe a third party's intellectual property rights, we could be required to obtain a license from such third party to continue developing and marketing our technology and drugs. However, we may not be able to obtain any required license on commercially reasonable terms, or at all. Even if we were able to obtain a license, it could be non-exclusive, thereby giving our competitors access to the same technologies licensed to us. We could be forced, including by court order, to cease commercializing the infringing technology or product. In addition, we could be found liable for monetary damages, including treble damages and attorneys' fees if we are found to have willfully infringed a patent. A finding of infringement could prevent us from commercializing our product candidates or force us to cease some or all of our business operations.

Competing products may also be sold in other countries in which our patent coverage might not exist or be as strong. If we lose a foreign patent lawsuit alleging our infringement of a competitor's patent, we could be prevented from marketing our products in one or more foreign countries. As a result, our ability to grow our business and compete in the market may be harmed.

Intellectual property litigation could cause us to spend substantial resources and distract our personnel from their normal responsibilities.

Even if resolved in our favor, litigation or other legal proceedings relating to intellectual property claims may cause us to incur significant expenses and could distract our technical and management personnel from their normal responsibilities.

In addition, there could be public announcements of the results of hearings, motions or other interim proceedings or developments. If securities analysts or investors perceive these results to be negative, it could hurt the price of our common stock. Such litigation or proceedings could substantially increase our operating losses and reduce the resources available for development activities or any future sales, marketing or distribution activities. We may not have sufficient financial or other resources to conduct such litigation or proceedings adequately. Some of our competitors may be able to sustain the costs of such litigation or proceedings more effectively than we can because of their greater financial resources. Uncertainties resulting from the initiation and continuation of patent litigation or other proceedings could compromise our ability to compete in the marketplace.

We may need to license intellectual property from third parties, and such licenses may not be available or may not be available on commercially reasonable terms.

A third party may hold intellectual property rights, including patent rights, which are important or necessary to the development of our product candidates. It may be necessary for us to use the patented or proprietary technology of third parties to commercialize our product candidates, in which case we would be required to obtain a license from these third parties. Such a license may not be available on commercially reasonable terms, or at all, and we could be forced to accept unfavorable contractual terms. If we are unable to obtain such licenses on commercially reasonable terms, our business could be harmed.

If we or our third-party licensors fail to comply with our obligations in our intellectual property licenses and funding arrangements with third parties, we could lose rights that are important to our business.

We are currently party to license agreements for technologies that we anticipate using in our product development activities. In the future, we may become party to licenses that are important for product development and commercialization. If we or our third-party licensors fail to comply with the obligations under current or future license and funding agreements, our counterparties may have the right to terminate these agreements, we may be forced to terminate these agreement or we may no longer effectively rely on any licenses to us under these agreements, in which event we might not be able to develop, manufacture or market any product or utilize any technology that is covered by these agreements or may face other penalties under the agreements. Such an occurrence could materially and adversely affect the value of a product candidate being developed under any such agreement or could restrict our drug discovery activities. Termination of these agreements or reduction or elimination of our rights under these agreements may result in our having to negotiate new or reinstated agreements with less favorable terms or cause us to lose our rights under these agreements, including our rights to important intellectual property or technology.

The scope of our intellectual property may be reduced or may need to be reduced due to third-party intellectual property claims.

Our competitors may have filed, and may in the future file, patent applications covering technology similar to ours. Any such patent application may have priority over our patent applications, which could further require us to obtain rights to issued patents covering such technologies. If another party has filed a U.S. patent application on inventions similar to ours that claims priority to an application filed prior to March 16, 2013, we may have to participate in an interference proceeding declared by the USPTO to determine priority of invention in the United States. The costs of these proceedings could be substantial, and it is possible that such efforts would be unsuccessful if, unbeknownst to us, the other party had independently arrived at the same or similar invention prior to our own invention, resulting in a loss of our U.S. patent position with respect to such inventions. In addition, changes enacted on March 16, 2013, to the U.S. patent laws under the Leahy-Smith Act resulted in the United States changing from a “first to invent” country to a “first to file” country. As a result, we may lose the ability to obtain a patent if another party files with the USPTO first and could become involved in proceedings before the USPTO to resolve disputes related to inventorship. We may also become involved in similar proceedings in other jurisdictions.

Furthermore, recent changes in U.S. patent law under the Leahy-Smith Act allow for post-issuance challenges to U.S. patents, including ex parte re-examinations, *inter partes* reviews and post-grant reviews. There is significant uncertainty as to how the new laws will be applied. If our U.S. patents are challenged using such procedures, we may not prevail, possibly resulting in altered or diminished claim scope or loss of patent rights altogether. Similarly, some countries, notably in Europe, also have post-grant opposition proceedings or nullity proceedings that can result in changes in scope or cancellation of patent claims.

We may be subject to claims by third parties asserting that we or our employees have misappropriated their intellectual property or claiming ownership of what we regard as our own intellectual property.

The FDA and foreign regulatory authorities closely regulate promotional materials and other promotional activities. Even if the FDA or foreign regulatory authorities initially approve product labeling that includes a description of our improved attribute claims, the FDA and foreign regulatory authorities may object to our marketing claims and product advertising campaigns. Failure to comply with the FDA's and comparable foreign regulatory authorities' promotional, marketing and advertising laws and regulations could lead to the issuance of warning letters, cyber letters, or untitled letters, adverse publicity, the requirement for dear-health-care-provider letters or other corrective information, fines and other monetary penalties, civil or criminal prosecution, including False Claims Act liability, restrictions on our operations and other operating requirements through consent decrees or corporate integrity agreements, debarment, exclusion from participation in federal health care programs and refusal of government contracts or future orders under existing contracts, among other consequences. Any of these consequences would harm the commercial success of our products.

Further, our promotional materials, statements and training methods must comply with the FDA's and comparable foreign regulatory authorities' prohibition of the promotion of unapproved, or off-label, use. Any regulatory approval that the FDA grants is limited to those specific diseases and indications for which a product is deemed to be safe and effective by FDA. Similar requirements apply in foreign jurisdictions. For example, the FDA-approved label for AZSTARYS is limited to the acute treatment of ADHD in patients 6 years of age and older, and the FDA-approved label for APADAZ is limited to the short-term (no more than 14 days) management of acute pain severe enough to require an opioid analgesic and for which alternative treatments are inadequate. Physicians may use our products off-label, as the FDA does not restrict or regulate a physician's independent choice of treatment within the practice of medicine. However, if the FDA or foreign regulatory authorities determine that our promotional materials, statements or training constitutes promotion of an off-label use, it could request that we modify our promotional materials, statements or training methods or subject us to regulatory or enforcement actions, such as the issuance of an untitled letter, a warning letter, injunction, seizure, civil fine, disgorgement of money, operating restrictions or criminal penalties. We may also be subject to actions by other governmental entities or private parties, such as the False Claims Act, civil whistleblower or "qui tam" actions. It is also possible that other federal, state or foreign enforcement authorities might take action if they consider our promotional or training materials to constitute promotion of an off-label use, which could result in significant fines or penalties under other statutory authorities, such as laws prohibiting false claims for reimbursement. In that event, our reputation could be damaged and adoption of the products could be impaired. In addition, the off-label use of our products may increase the risk of product liability claims. Product liability claims are expensive to defend and could divert our management's attention, result in substantial damage awards against us and harm our reputation.

Any trademarks we may obtain may be infringed or successfully challenged, resulting in harm to our business.

We expect to rely on trademarks as one means to distinguish our product candidates that are approved for marketing from the products of our competitors. We have registered trademarks, including those for LAT, Zevra, and OLPRUVA. In addition, we have solicited and applied for trademarks for the Zevra logo and several potential trade names and logos for future product candidates. Third parties may oppose or attempt to cancel our trademark applications or trademarks, or otherwise challenge our use of the trademarks. If our trademarks are successfully challenged, we could be forced to rebrand our products, which could result in loss of brand recognition and could require us to devote resources to advertising and marketing new brands. Our competitors may infringe our trademarks and we may not have adequate resources to enforce our trademarks.

If we are unable to protect the confidentiality of our trade secrets, our business and competitive position would be harmed.

In addition to seeking patent and trademark protection for our product candidates, we also rely on trade secrets, including unpatented show-how, know-how, technology and other proprietary information, to maintain our competitive position. We seek to protect our trade secrets, in part, by entering into non-disclosure and confidentiality agreements with parties who have access to them, such as our employees, corporate collaborators, outside scientific collaborators, contract manufacturers, consultants, advisors and other third parties. We also enter into confidentiality and invention or patent assignment agreements with our employees and consultants. Despite these efforts, any of these parties may breach the agreements and disclose our proprietary information, including our trade secrets. Further, we may need to share our trade secrets and confidential know-how with future business partners, collaborators, contractors and others located in countries at heightened risk of theft of trade secrets, including through direct intrusion by private parties or actors in other countries, and those affiliated with or controlled by state actors.

Monitoring unauthorized uses and disclosures of our intellectual property, including our trade secrets, is difficult, and we do not know whether the steps we have taken to protect our intellectual property will be effective. In addition, we may not be able to obtain adequate remedies for any such breaches. Enforcing a claim that a party illegally disclosed or misappropriated a trade secret is difficult, expensive and time-consuming, and the outcome is unpredictable. In addition, some courts inside and outside the United States are less willing or unwilling to protect trade secrets.

Moreover, our competitors may independently develop or reverse engineer knowledge, methods, show-how and know-how equivalent to our trade secrets. Competitors could purchase our products and replicate some or all of the competitive advantages we derive from our development efforts for technologies on which we do not have patent protection. If any of our trade secrets were to be lawfully obtained or independently developed by a competitor, we would have no right to prevent them, or those to whom they communicate such trade secrets, from using that technology or information to compete with us. If any of our trade secrets were to be disclosed to or independently developed by a competitor, our competitive position would be harmed.

We may not be able to protect our intellectual property rights throughout the world.

Patents are of national or regional effect, and filing, prosecuting and defending patents on all of our products and technologies throughout the world would be prohibitively expensive. As such, our intellectual property rights in some countries outside the United States can be less extensive than those in the United States and we may not be able to prevent third parties from practicing our inventions in all countries outside the United States, or from selling or importing products made using our inventions in and into the United States or other jurisdictions. Competitors may use our technologies in jurisdictions where we have not obtained patent protection to develop their own products or technology and may export otherwise infringing products or technology to territories where we have patent protection, but enforcement rights are not as strong as those in the United States. These products may compete with our products and technologies, and our patents or other intellectual property rights may not be effective or sufficient to prevent them from competing. Further, the legal systems of certain countries, particularly certain developing countries, do not favor the enforcement of patents and other intellectual property protection, particularly those relating to pharmaceuticals or biologics, which could make it difficult for us to stop the infringement of our patents or marketing of competing products in violation of our proprietary rights generally. Proceedings to enforce our patent rights in other jurisdictions could result in substantial costs and divert our efforts and attention from other aspects of our business, could put our patents at risk of being invalidated or interpreted narrowly and our patent applications at risk of not issuing and could provoke third parties to assert claims against us. We may not prevail in any such lawsuits that we initiate and the damages and other remedies awarded, if any, may not be commercially meaningful. Similarly, if our trade secrets are disclosed in another jurisdiction, competitors worldwide could have access to our proprietary information and we may be without satisfactory recourse. Such disclosure could have a material adverse effect on our business. Moreover, our ability to protect and enforce our intellectual property rights may be adversely affected by unforeseen changes in intellectual property laws of other countries. In addition, certain developing countries, including China and India, have compulsory licensing laws under which a patent owner may be compelled to grant licenses to third parties. In those countries, we and our licensors may have limited remedies if patents are infringed or if we or our licensors are compelled to grant a license to a third party, which could materially diminish the value of those patents. In addition, many countries limit the enforceability of patents against government agencies or government contractors. This could limit our potential revenue opportunities. Accordingly, our efforts to enforce our intellectual property rights around the world may be inadequate to obtain a significant commercial advantage from the intellectual property that we develop or license.

In 2012, the European Patent Package, or EU Patent Package, regulations were passed with the goal of providing a single pan-European Unitary Patent and a new European Unified Patent Court, or UPC, for litigation involving European patents. Implementation of the EU Patent Package will likely occur in the first half of 2023. Under the UPC, all European patents, including those issued prior to ratification of the European Patent Package, will by default automatically fall under the jurisdiction of the UPC. The UPC will provide our competitors with a new forum to centrally revoke our European patents, and allow for the possibility of a competitor to obtain pan-European injunctions. It will be several years before we will understand the scope of patent rights that will be recognized and the strength of patent remedies that will be provided by the UPC. Under the EU Patent Package as currently proposed, we will have the right to opt our patents out of the UPC over the first seven years of the court's existence, but doing so may preclude us from realizing the benefits of the new unified court.

Our decision to seek approval of our product candidates under the 505(b)(2) NDA pathway, if available, may increase the risk that patent infringement suits are filed against us, which would delay the FDA's approval of such product candidates.

Regarding any NDA that we may submit under the 505(b)(2) NDA pathway, if there are patents that claim the approved drug contained in our product candidates and referenced in our 505(b)(2) NDA, we must certify to the FDA and notify the patent holder that any patents listed for the approved drug in the FDA's Orange Book publication are invalid, unenforceable or will not be infringed by the manufacture, use or sale of our prodrug. If a patent infringement lawsuit is filed against us within 45 days of its receipt of notice of our certification, the FDA is automatically prevented from approving our 505(b)(2) NDA until the earliest of 30 months, expiration of the patent, settlement of the lawsuit or a court decision in the infringement case that is favorable to us, or such shorter or longer period as may be ordered by a court. Such actions are routinely filed by patent owners. Accordingly, we may invest considerable time and expense in the development of our product candidates only to be subject to significant delay and patent litigation before our product candidates may be commercialized. We may not be successful in defending any patent infringement claim. Even if we are found not to infringe, or a plaintiff's patent claims are found invalid or unenforceable, defending any such infringement claim would be expensive and time-consuming, and would delay launch of our product candidates and distract management from their normal responsibilities.

Risks Related to the Commercialization of Our Partnered Products and Product Candidates

If we are unable to establish sales, marketing and distribution capabilities for our approved products or product candidates, if approved, we may not be successful in commercializing any approved product candidate in the United States.

While we entered into the AZSTARYS License Agreement to establish a collaboration for the commercialization of AZSTARYS and any of our product candidates which are subject to such agreement, we currently have limited marketing and sales experience. In order to commercialize OLPRUVA for oral suspension in the U.S. for the treatment of certain patients with UCDs we have added marketing, sales, medical affairs, distribution, managerial and other non-technical capabilities, or have made arrangements with third parties to perform these services. For any of our other product candidates that receive marketing approval we may have to augment our commercial capabilities, or make arrangements with third parties to perform additional services, and we may not be successful in doing any of the foregoing. Building and maintaining a targeted specialty sales force is expensive and time consuming. Any failure or delay in the development of our internal sales, marketing and distribution capabilities would adversely impact our commercialization efforts. We may choose to collaborate with third parties that have their own sales forces and established distribution systems, in lieu of or to augment any sales force and distribution systems we may create. If we are unable to enter into collaborations with third parties for the commercialization of approved product candidates, if any, on acceptable terms or at all, or if any such collaborator does not devote sufficient resources to the commercialization of our product or otherwise fails in commercialization efforts, we may not be able to successfully commercialize our product candidates that receive marketing approval. If we are not successful in commercializing our product candidates that receive marketing approval, either on our own or through collaborations with one or more third parties, our potential future revenue will be materially and adversely impacted.

Establishing our own sales, marketing and distribution capabilities, involves a number of risks. For example, recruiting and training a sales force is expensive and time consuming and could delay any product launch. If the commercial launch of a product candidate for which we recruit a sales force and establish marketing capabilities is delayed or does not occur for any reason, we would have prematurely or unnecessarily incurred these commercialization expenses. This may be costly, and our investment would be lost if we cannot retain or reposition our sales and marketing personnel.

Factors that may inhibit our efforts to commercialize our approved products and product candidates on our own include:

- our inability to recruit, train and retain adequate numbers of effective sales and marketing personnel;
- our inability to access government and commercial health plan formularies or secure preferred coverage and adequate reimbursement levels;
- the inability of sales personnel to obtain access to physicians or achieve adequate numbers of physicians to prescribe any future prodrug products;
- the lack of complementary drugs to be offered by sales personnel, which may put us at a competitive disadvantage relative to companies with more extensive product lines;
- liability for personnel, including sales personnel, failing to comply with applicable legal requirements; and
- costs associated with maintaining compliance with the FDA's marketing and promotional requirements, including ongoing training and monitoring, as well as unforeseen costs and expenses associated with creating an independent sales and marketing organization.

If we decide to enter into arrangements with third parties to perform these services for certain of our product candidates, our product revenue and our profitability, if any, are likely to be lower than if we were to sell, market and distribute any product candidates that we develop ourselves. In addition, we may not be successful in entering into arrangements with third parties to sell, market and distribute our product candidates in the future, or may be unable to do so on terms that are favorable to us. We likely will have little control over such third parties, including Corium, and any of them may fail to devote the necessary resources and attention to sell and market our approved products, or any of our product candidates, if approved, effectively. Further, we may be liable for conduct of third parties, including Corium, acting on our behalf, including failure to comply with legal requirements applicable to sales and marketing of our product or product candidates, if approved. If we do not establish sales, marketing and distribution capabilities successfully, either on our own or in collaboration with third parties, we will not be successful in commercializing our approved products, or any of our product candidates, if approved.

Our approved products, or any of our product candidates that may receive marketing approval, may fail to achieve the degree of market acceptance by physicians, patients, third-party payors and others in the medical community necessary for commercial success.

Our approved products, or any of our product candidates that may receive marketing approval, may fail to gain sufficient market acceptance by physicians, patients, third-party payors and others in the medical community. If our approved products, or any of our product candidates, if approved for commercial sale, do not achieve an adequate level of market acceptance, they may not generate significant product revenue and we may not become profitable. The degree of market acceptance of our approved products, or any of our product candidates if approved for commercial sale, will depend on a number of factors, including:

- the efficacy and potential advantages compared to alternative treatments, including less expensive generic treatments;
- the ability to obtain differentiating claims in the labels for most of our product candidates;
- our ability to offer our prodrug products for sale at competitive prices;
- the clinical indications for which our product candidates are approved;
- the convenience and ease of administration compared to alternative treatments;
- the willingness of the target patient population to try new therapies and of physicians to prescribe these therapies;
- the cost of treatment in relation to alternative treatments;
- the steps that prescribers and dispensers must take, since our approved products are, and we expect that most of our product candidates are likely going to be considered controlled substances, as well as the perceived risks based upon their controlled substance status;
- the ability to manufacture our product in sufficient quantities and yields;
- the strength of marketing and distribution support;
- the availability of third-party coverage and adequate reimbursement or willingness of patients to pay out of pocket in the absence of third-party coverage;
- the prevalence and severity of any side effects;
- any potential unfavorable publicity;
- any restrictions on the use, sale or distribution of our approved products or any of our product candidates, including through REMS; and
- any restrictions on the use of our prodrug products together with other medications.

We face substantial competition, which may result in others discovering, developing or commercializing products before or more successfully than we do.

Our industry is characterized by rapidly advancing technologies, intense competition and a strong emphasis on proprietary products. We will face competition and potential competition from a number of sources, including pharmaceutical and biotechnology companies, specialty pharmaceutical companies, generic drug companies, drug delivery companies and academic and research institutions. Our competitors may develop or market drugs that are more effective, more convenient, more widely used and less costly or have a better safety profile than our products or product candidates and these competitors may also have significantly more resources than us and be more successful than us in manufacturing and marketing their products.

Currently, there are no approved drugs in the United States for the treatment of NPC. We consider our most direct competitor with respect to arimoclomol to be Zavesca (miglustat), which was originally developed by Actelion Pharmaceuticals and is now owned by Johnson & Johnson. Miglustat is currently approved for the treatment of NPC in Europe, Canada, Australia, New Zealand, and several countries in Asia and South America as ZAVESCA and in Japan as BRAZAVES.

If approved, we intend for KP1077 to compete against XYWAV®, marketed by Jazz Pharmaceuticals' , and potentially with other products that are currently in development for the treatment of IH, including Harmony Biosciences' WAKIX. KP1077 could face potential competition from any products for the treatment of IH that are currently in or which may enter into clinical development.

AZSTARYS currently competes against currently marketed, branded and generic methylphenidate products for the treatment of ADHD. Some of these currently marketed products include CONCERTA®, marketed by J&J Innovative Medicines (formerly Jannsen), QUELBREE®, marketed by Supernus Pharmaceuticals, Inc., QUILLIVANT XR®and QUILLICHEW ER® marketed by Tris Pharma, RITALIN, FOCALIN® and FOCALIN XR®, marketed by Novartis AG, METADATE CD®, marketed by UCB SA, DAYTRANA®, marketed by Noven Therapeutics, LLC, Neos Therapeutics' CONTEMPLA XR-ODT®, marketed Aytu BioScience, Inc., JORNAY PM®, Ironshore Pharmaceuticals, Inc., and ADHANSIA XR®, marketed by Adlon Therapeutics,'. In addition, AZSTARYS will face potential competition from any other methylphenidate products for the treatment of ADHD that are currently in, or which may enter into clinical development.

Our potential competitors and the related stage of development for their product candidates in our target indications for OLPRUVA (sodium phenylbutyrate) for oral suspension and celiprolol include for UCDs, Horizon Pharma plc / Immedica Group (Marketed) and Medunik USA (Marketed), and for vEDS, Aytu BioPharma (AR101/enzastaurin development indefinitely suspended October 2022)

Many of our potential competitors have substantially greater financial, technical and human resources than we do, as well as more experience in the development of product candidates, obtaining FDA and other regulatory approvals of products and the commercialization of those products. Consequently, our competitors may develop products for indications we are pursuing or may pursue in the future, and such competitors' products may be more effective, better tolerated and less costly than our product candidates. Our competitors may also be more successful in manufacturing and marketing their products than we are. We will also face competition in recruiting and retaining qualified personnel and establishing clinical trial sites and patient enrollment in clinical trials.

Our competitors also may obtain FDA or other regulatory approval for their product candidates more rapidly than we may obtain approval for ours, which could result in our competitors establishing a strong market position before we are able to enter the market. If the competitor's product were similar to our product candidates, we may be required to seek approval via alternative pathways, such as the ANDA, which is used for the development of generic drug products. We may also be blocked from product marketing by periods of patent protection or regulatory exclusivity.

In addition, our ability to compete may be affected in many cases by insurers or other third-party payors seeking to encourage the use of generic drugs or giving drugs with improved attributes sufficient weight in a comparative clinical cost effectiveness analysis. For some of the indications that we are pursuing, drugs used off-label serve as cheaper alternatives to our product candidates. Their lower prices could result in significant pricing pressure, even if our product candidates are otherwise viewed as a preferable therapy. Additional drugs may become available on a generic basis over the coming years.

Mergers and acquisitions in the pharmaceutical and biotechnology industries may result in even more resources being concentrated among a smaller number of our competitors. Smaller and other early-stage companies may also prove to be significant competitors, particularly through collaborative arrangements with large and established companies.

Consequently, our competitors may develop products for the treatment of ADHD, pain, UCDs, or for other indications pursue or we may pursue in the future, and such competitors' products may be more effective, better tolerated and less costly than our product candidates. Our competitors may also be more successful in manufacturing and marketing their products than we are. We will also face competition in recruiting and retaining qualified personnel and establishing clinical trial sites and subject enrollment in clinical trials.

We may not be able to obtain either five-year FDA regulatory exclusivity as a new chemical entity or three-year FDA regulatory exclusivity.

The FDA provides periods of regulatory exclusivity following their approval of an NDA, which provide the holder of an approved NDA limited protection from new competition in the marketplace for the innovation represented by its approved drug. Five-year exclusivity precludes approval of 505(b)(2) applications or ANDAs by delaying the submission or approval of the application, while three-year exclusivity precludes the approval of the application. We intend to seek new chemical entity, or NCE, status for any of our prodrug product candidates as appropriate. Five years of exclusivity are available to NCEs following the approval of an NDA by the FDA. An NCE is a drug that contains no active moiety that has been approved by the FDA in any other NDA. If a product is not eligible for the NCE exclusivity, it may be eligible for three years of exclusivity. Three-year exclusivity is available to the holder of an NDA, including a 505(b)(2) NDA, for a particular condition of approval, or change to a marketed product, such as a new formulation for a previously approved product, if one or more new clinical trials, other than bioavailability or bioequivalence trials, were essential to the approval of the application and were conducted or sponsored by the applicant.

There is a risk that the FDA may disagree with any claim that we may make that any of our prodrug product candidates are NCEs and therefore entitled to five-year exclusivity. The FDA may also take the view that the studies that we are conducting are not clinical trials, other than bioavailability and bioequivalence studies, that are essential to approval and therefore do not support three-year exclusivity. Further, to the extent that the basis for exclusivity is not clear, the FDA may determine to defer a decision until it receives an application which necessitates a decision.

If we do obtain either five or three years of exclusivity, such exclusivity will not block all potential competitors from the market. Competitors may be able to obtain approval for similar products with different forms of competitive differentiating mechanisms or may be able to obtain approval for similar products without a competitive differentiating mechanism.

Even if we or our collaborators are able to commercialize our approved products, or any of our product candidates, if approved, they may be subject to unfavorable pricing regulations, third-party coverage and reimbursement policies.

The successful commercialization of our approved products, and any of our product candidates for which marketing approval is obtained will depend, in part, on the extent to which coverage and adequate reimbursement for AZSTARYS, OLPRUVA, or any of our product candidates for which marketing approval is obtained, will be available from government payor programs at the federal and state levels, including Medicare and Medicaid, private health insurers and managed care plans and other third-party payors. Government authorities and other third-party payors decide which medical products they will pay for and establish reimbursement levels, including co-payments. A trend in the U.S. healthcare industry and elsewhere is cost containment. Government authorities and other third-party payors have attempted to control costs by limiting coverage and the amount of reimbursement for particular medical products. Increasingly, third-party payors are requiring that drug companies provide them with predetermined discounts from list prices and are challenging the prices charged for drugs and products. Coverage and reimbursement may not be available for any product that we commercialize and, even if these are available, the level of reimbursement may not be satisfactory. Inadequate reimbursement levels may adversely affect the demand for, or the price of, our approved products, or any of our product candidates for which we obtain marketing approval. Obtaining and maintaining adequate reimbursement for our prodrug products may be difficult. We may be required to conduct expensive pharmacoeconomic studies to justify coverage and reimbursement or the level of reimbursement relative to other therapies. Moreover, the trend has been for government and commercial health plans and their pharmacy benefit managers to commoditize drug products through therapeutic equivalence determinations, making formulary decisions based on cost. If coverage and adequate reimbursement are not available or reimbursement is available only at limited levels, we may not be able to successfully commercialize our approved products or commercialize any of our product candidates for which marketing approval is obtained.

There may be significant delays in obtaining coverage and reimbursement for newly approved prodrug products, and coverage may be more limited than the indications for which the product is approved by the FDA or similar regulatory authorities outside the United States. Moreover, eligibility for coverage and reimbursement does not imply that a product will be paid for in all cases or at a rate that covers our costs, including research, development, manufacture, sale and distribution expenses. Interim reimbursement levels for new prodrug products, if applicable, may also not be sufficient to cover our costs and may not be made permanent. Reimbursement rates may vary according to the use of the product and the clinical setting in which it is used, may be based on reimbursement levels already set for lower cost drugs and may be incorporated into existing payments for other services. Net prices for prodrug products may be reduced by mandatory discounts or rebates required by government healthcare programs or private payors and by any future relaxation of laws that presently restrict imports of drugs from countries where they may be sold at lower prices than in the United States. Private third-party payors often rely upon Medicare coverage policy and payment limitations in setting their own reimbursement policies. Except for certain government health care programs, such as the Department of Defense's TRICARE Uniform Formulary, no uniform policy requirement for coverage and reimbursement for drug products exists among third-party payors in the United States. Even state Medicaid programs have their own preferred drug lists that may disadvantage non-preferred brand drugs. Therefore, coverage and reimbursement can differ significantly from payor to payor. As a result, the coverage determination process is often a time-consuming and costly process that will require us to provide scientific and clinical support for the use of our products to each payor separately, with no assurance that coverage and adequate reimbursement will be applied consistently or obtained at all. Our inability to promptly obtain coverage and adequate reimbursement rates from both government-funded and private payors for any approved prodrug products that we develop could significantly harm our operating results, our ability to raise capital needed to commercialize prodrugs and our overall financial condition.

The regulations that govern marketing approvals, pricing, coverage and reimbursement for new drugs vary widely from country to country. Current and future legislation may significantly change the approval requirements in ways that could involve additional costs and cause delays in obtaining approvals. Some countries require approval of the sale price of a product before it can be marketed. In many countries, the pricing review period begins after marketing or product licensing approval is granted. In some foreign markets, prescription pharmaceutical pricing remains subject to continuing governmental control even after initial approval is granted. As a result, we might obtain marketing approval for a product in a particular country, but then be subject to price regulations that delay commercial launch of the product, possibly for lengthy time periods, and negatively impact the revenue able to be generated from the sale of the product in that country. Adverse pricing limitations may hinder our ability to recoup our investment in one or more product candidates, even if our product candidates obtain marketing approval.

There can be no assurance that our approved products, or any of our product candidates, if they are approved for sale in the United States or in other countries, will be considered medically reasonable and necessary for a specific indication, that they will be considered cost-effective by third-party payors, that coverage or an adequate level of reimbursement will be available, or that third-party payors' reimbursement policies will not adversely affect the ability to sell our approved products, or our ability to sell any of any of our product candidates profitably if they are approved for sale.

We may be subject to enforcement action if we engage in improper marketing or promotion of our products.

The FDA closely regulates promotional materials and other promotional activities. Even if the FDA initially approves product labeling that includes a description of our improved attribute claims, the FDA may object to our marketing claims and product advertising campaigns. Failure to comply with the FDA's promotional, marketing and advertising laws and regulations could lead to the issuance of warning letters, cyber letters, or untitled letters, adverse publicity, the requirement for dear-health-care-provider letters or other corrective information, fines and other monetary penalties, civil or criminal prosecution, including False Claims Act liability, restrictions on our operations and other operating requirements through consent decrees or corporate integrity agreements, debarment, exclusion from participation in federal health care programs and refusal of government contracts or future orders under existing contracts, among other consequences. Any of these consequences would harm the commercial success of our products.

Further, our promotional materials, statements and training methods must comply with the FDA's prohibition of the promotion of unapproved, or off-label, use. Any regulatory approval that the FDA grants is limited to those specific diseases and indications for which a product is deemed to be safe and effective by FDA. For example, the FDA-approved label for AZSTARYS is limited to the acute treatment of ADHD in patients 6 years of age and older and the FDA approved label for OLPRUVA is limited to oral suspension in the U.S. for the treatment of certain patients with UCDs involving deficiencies of CPS, OTC, or AS. Physicians may use our products off-label, as the FDA does not restrict or regulate a physician's independent choice of treatment within the practice of medicine. However, if the FDA determines that our promotional materials, statements or training constitutes promotion of an off-label use, it could request that we modify our promotional materials, statements or training methods or subject us to regulatory or enforcement actions, such as the issuance of an untitled letter, a warning letter, injunction, seizure, civil fine, disgorgement of money, operating restrictions or criminal penalties. We may also be subject to actions by other governmental entities or private parties, such as the False Claims Act, civil whistleblower or "qui tam" actions. It is also possible that other federal, state or foreign enforcement authorities might take action if they consider our promotional or training materials to constitute promotion of an off-label use, which could result in significant fines or penalties under other statutory authorities, such as laws prohibiting false claims for reimbursement. In that event, our reputation could be damaged and adoption of the products could be impaired. In addition, the off-label use of our products may increase the risk of product liability claims. Product liability claims are expensive to defend and could divert our management's attention, result in substantial damage awards against us and harm our reputation.

Product liability lawsuits against us could cause us to incur substantial liabilities and to limit commercialization of our approved products, or any of our product candidates that we may develop.

We face an inherent risk of product liability exposure related to the testing of our product candidates in human clinical trials and will face an even greater risk as our approved products, and any of our product candidates that may be approved in the future, are commercialized. This includes the risk that our products may be misused. For example, our approved products do, and we anticipate that any of our product candidates we may choose to develop in the future, if approved, may carry, a boxed warning regarding lethality if our oral tablets or capsules are prepared for injection and hepatotoxicity, as is commonly done by abusers of opioids. If we cannot successfully defend ourselves against claims that our product candidates or products caused injuries, we will incur substantial liabilities on behalf of ourselves. Regardless of merit or eventual outcome, liability claims may result in:

- decreased demand for our approved products, and any of our product candidates that we may develop;
- injury to our reputation and significant negative media attention;
- termination of clinical trial sites or entire trial programs;
- withdrawal of clinical trial participants;
- initiation of investigations by regulators;
- significant costs to defend the related litigation;
- a diversion of management's time and our resources;
- substantial monetary awards paid to trial participants or patients;
- product recalls, withdrawals or labeling revisions and marketing or promotional restrictions;
- loss of revenue;
- reduced resources of our management to pursue our business strategy; and
- the inability to successfully commercialize any of our approved products, or any of our product candidates that might be approved in the future.

We currently hold $10.0 million in product liability insurance coverage in the aggregate, with a per incident limit of $10.0 million, which may not be adequate to cover all liabilities that we may incur. We may need to increase our insurance coverage as we expand our clinical trials or upon commencement of commercialization of any product approved in the future. Insurance coverage is increasingly expensive. We may not be able to maintain insurance coverage at a reasonable cost or in an amount adequate to satisfy any liability that may arise.

A variety of risks associated with international operations could materially adversely affect our business.

We expect to engage in significant cross-border activities, and we will be subject to risks related to international operations, including:

- different regulatory requirements for maintaining approval of drugs in foreign countries;
- differing payor reimbursement regimes, governmental payors or patient self-pay systems and price controls;
- reduced protection for contractual and intellectual property rights in some countries;
- unexpected changes in tariffs, trade barriers and regulatory requirements;
- economic weakness, including inflation, labor shortages, supply chain shortages, or other economic or political uncertainties or instability in particular foreign economies and markets;
- compliance with tax, employment, immigration and labor laws for employees living or traveling abroad;
- foreign currency fluctuations, which could result in increased operating expenses and reduced revenue, and other obligations incident to doing business in another country;
- workforce uncertainty in countries where labor unrest is more common than in North America;
- tighter restrictions on privacy and the collection and use of patient and clinical trial participant data; and
- business interruptions resulting from geopolitical actions, including war and terrorism, or natural disasters including earthquakes, typhoons, floods and fires.

Risks Related to Regulatory Approval of Our Product Candidates and Other Legal Compliance Matters

Failure to obtain marketing approval in international jurisdictions would prevent our approved products, and any of our other product candidates from being marketed abroad.

In order to market and sell our products in the European Union and any other jurisdictions, we must obtain separate marketing approvals and comply with numerous and varying regulatory requirements. The approval procedure varies among countries and can involve additional testing. The time required to obtain approval may differ substantially from that required to obtain FDA approval. The regulatory approval process outside the United States generally includes all of the risks associated with obtaining FDA approval. In addition, in many countries outside the United States, it is required that the product be approved for reimbursement before the product can be approved for sale in that country. We may not obtain approvals from regulatory authorities outside the United States on a timely basis, if at all. Approval by the FDA does not ensure approval by regulatory authorities in other countries or jurisdictions, and approval by one regulatory authority outside the United States does not ensure approval by regulatory authorities in other countries or jurisdictions or by the FDA. However, failure to obtain approval in one jurisdiction may impact our ability to obtain approval elsewhere. We may not be able to file for marketing approvals and may not receive necessary approvals to commercialize our products in any market.

A variety of risks associated with marketing our approved products and any of our product candidates, if approved, internationally, could affect our business.

We may seek regulatory approval for our approved products and any of our product candidates, if approved, outside of the United States and, accordingly, we expect that we will be subject to additional risks related to operating in foreign countries if we obtain the necessary approvals, including:

- differing regulatory requirements in foreign countries;
- the potential for so-called parallel importing, which is what happens when a local seller, faced with high or higher local prices, opts to import goods from a foreign market with low or lower prices rather than buying them locally;
- unexpected changes in tariffs, trade barriers, price and exchange controls and other regulatory requirements;
- economic weakness, including inflation, labor shortages, supply chain shortages, or other economic or political uncertainties or instability in particular foreign economies and markets;
- compliance with tax, employment, immigration and labor laws for employees living or traveling abroad;
- foreign taxes, including withholding of payroll taxes;
- foreign currency fluctuations, which could result in increased operating expenses and reduced revenue, and other obligations incident to doing business in another country;
- difficulties staffing and managing foreign operations;
- workforce uncertainty in countries where labor unrest is more common than in the United States;
- potential liability under the Foreign Corrupt Practices Act of 1977 or comparable foreign regulations;
- challenges enforcing our contractual and intellectual property rights, especially in those foreign countries that do not respect and protect intellectual property rights to the same extent as the United States;
- production shortages resulting from any events affecting raw material supply or manufacturing capabilities abroad; and
- business interruptions resulting from geo-political actions, including war and terrorism, such as the current situation with Ukraine and Russia.

These and other risks associated with our international operations may compromise our ability to achieve or maintain profitability.

Our approved products are, and any of our product candidates for which we obtain marketing approval will remain subject to significant post-marketing regulatory requirements and oversight.

Our approved products are, and any of our product candidates for which we obtain marketing approval could be, subject to significant post-marketing regulatory requirements and oversight, including the submission of reports to regulatory authorities and surveillance to monitor the safety and efficacy of the product, significant limitations related to use restrictions for specified age groups, warnings, precautions or contraindications, and burdensome post-approval study or risk management requirements.

For example, we are required to conduct pediatric studies related to AZSTARYS to evaluate its safety and effectiveness for the claimed indication in pediatric patients. Under the AZSTARYS License Agreement, Corium will be responsible for these regulatory activities going forward, and we cannot guarantee they will be complied with.

For OLPRUVA for oral suspension in the U.S. for the treatment of certain patients with UCDs, the manufacturing processes, labeling, packaging, distribution, adverse event reporting, storage, advertising, promotion and recordkeeping for the product will be subject to extensive and ongoing regulatory requirements. These requirements include submissions of safety and other post-marketing information and reports, registration, as well as continued compliance with cGMP and comparable foreign regulations and GCP for any clinical trials that we conduct post-approval.

In addition, the manufacturing processes, labeling, packaging, distribution, adverse event reporting, storage, advertising, promotion, import, export and recordkeeping for any approved drugs are subject to extensive and ongoing regulatory requirements. These requirements include submissions of safety and other post-marketing information and reports, registration and listing requirements, payment of substantial annual product and establishment fees, labeling requirements, promotional, marketing and advertising requirements, requirements related to further development, packaging, storage and distribution requirements, cGMP requirements relating to manufacturing, quality control, quality assurance and corresponding maintenance of records and documents, requirements regarding the distribution of samples to physicians and recordkeeping. If there are any modifications to the drug, including changes in indications, labeling, manufacturing processes or facilities, or new safety issues arise, a new or supplemental NDA, a post-implementation notification or other reporting may be required or requested depending on the change, which may require additional data or additional preclinical studies and clinical trials.

Our approved products are, and if marketing approval of any of our product candidates is granted, such product candidates may be, subject to limitations on the indicated uses for which the product may be marketed or to the conditions of approval, including the requirement to implement a REMS, which could involve requirements for, among other things, a medication guide, special training for prescribers and dispensers, and patient registries. For example, in September 2018, the FDA approved the Opioid Analgesic REMS for ER/LA and IR opioids as one strategy among multiple national and state efforts to reduce the risk of abuse, misuse, addiction, overdose, and deaths due to prescription opioid analgesics. We anticipate that any opioid product candidates that we may choose to develop in the future, if approved by the FDA, are likely to also be subject to a REMS requirement. In addition, the FDA may also impose requirements for costly post-marketing studies or clinical trials and surveillance to monitor the safety or efficacy of the product.

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Products may be approved with a label that limits their approved uses, including more limited subject populations, than we request, and regulatory authorities may require that contraindications, warnings or precautions be included in the product labeling, including a boxed warning, or may approve a product candidate with a label that does not include the labeling claims necessary or desirable for the successful commercialization of that product candidate, which could limit sales of the product. For instance, the label for AZSTARYS contains black box warnings regarding the risks of abuse and dependence.

Violations of the FFDCA relating to the promotion of prescription drugs may lead to a number of actions and penalties, including warning letters, cyber letters, or untitled letters, adverse publicity, the requirement for dear-health-care-provider letters or other corrective information, fines and other monetary penalties, civil or criminal prosecution, including False Claims Act liability, restrictions on our operations and other operating requirements through consent decrees or corporate integrity agreements, debarment, exclusion from participation in federal health care programs and refusal of government contracts or future orders under existing contracts, among other consequences.

In addition, later discovery of previously unknown adverse events or other problems with our prodrug products, including those related to manufacturers or manufacturing processes, or failure to comply with regulatory requirements, may have negative consequences, including:

- adverse inspectional findings;
- restrictions on such prodrug products, distribution, manufacturers or manufacturing processes;
- restrictions on the labeling or marketing of a drug;
- additional warnings or other restrictions on the product's indicated use, label, or marketing;
- issuance of safety alerts, dear-healthcare-provider letters, press releases or other communications containing warnings regarding the product;
- requirement to establish or modify a REMS or similar risk management programs;
- requirement to conduct post-marketing studies or surveillance;
- restrictions on drug distribution or use;
- requirements to conduct post-marketing studies or clinical trials;
- warning letters;
- recall or withdrawal of the prodrug products from the market;
- refusal to approve pending applications or supplements to approved applications that we submit and other delays;
- clinical holds, or the suspension or termination of ongoing clinical trials;
- fines, restitution or disgorgement of profits or revenue;
- suspension or withdrawal of marketing approvals or other permits or voluntary suspension of marketing;
- refusal to permit the import or export of our prodrug products;
- reputational harm;
- refusal of government contracts or future orders under existing contracts, exclusion from participation in federal health care programs, and corporate integrity agreements;
- product seizure or detention; or
- injunctions or the imposition of civil or criminal penalties, including False Claims Act liability.

Non-compliance with European Union requirements regarding safety monitoring or pharmacovigilance, and with requi rements related to the development of drugs for the pediatric population, can also result in significant financial penalties. Similarly, failure to comply with the European Union's requirements regarding the protection of personal information can also lead to significant penalties and sanctions.

The occurrence of any event or penalty described above may inhibit our ability to commercialize our products and generate revenue, and could require us to expend significant time and resources in response and could generate negative publicity. In addition, the FDA's and other regulatory authorities' policies may change, and additional government regulations may be enacted that could impair our business. If we are slow or unable to adapt to changes in existing requirements or the adoption of new requirements or policies, or if we are not able to maintain regulatory compliance, we may be subject to enforcement action, and we may not achieve or sustain profitability.

Our employees, independent contractors, principal investigators, CROs, consultants, commercial collaborators, contract manufacturers, service providers and other vendors may engage in misconduct or other improper activities, including non-compliance with regulatory standards and requirements.

We are exposed to the risk of misconduct by employees and independent contractors, such as principal investigators, CROs, consultants, commercial collaborators, contract manufacturers, service providers and other vendors. Such misconduct could include failures to comply with FDA or comparable foreign regulations, to provide accurate information to the FDA, or comparable foreign authorities, to comply with manufacturing standards that we have established or that are established by regulation, to comply with federal and state contracting and healthcare fraud and abuse laws, to report drug pricing, financial information or data accurately or to disclose unauthorized activities to us. In particular, sales, marketing and other business arrangements in the healthcare industry are subject to extensive laws intended to prevent fraud, kickbacks, self-dealing and other abusive practices. These laws may restrict or prohibit a wide range of business activities, including, but not limited to, research, manufacturing, distribution, pricing, discounting, marketing, advertising and promotion, sales commissions, customer incentive programs and other business arrangements. Employee and independent contractor misconduct could also involve the improper use of individually identifiable information, including, without limitation, information obtained in the course of clinical trials, which could result in regulatory sanctions and serious harm to our reputation. In addition, federal procurement laws impose substantial penalties for misconduct in connection with government contracts and require certain contractors to maintain a code of business ethics and conduct and self-disclose credible evidence of False Claims Act violations.

It is not always possible to identify and deter employee and independent contractor misconduct, and any precautions we take to detect and prevent improper activities may not be effective in controlling unknown or unmanaged risks or losses or in protecting us from governmental investigations or other actions or lawsuits stemming from a failure to be in compliance with such laws. If any such actions are instituted against us, those actions could have a significant impact on our business, including the imposition of warning letters, untitled letters, cyber letters, seizure or recall of products, injunctions, withdrawal of product approval or other permits, clinical holds and termination of clinical trials, FDA or foreign regulatory authorities refusal to approve pending applications, product detentions, FDA or DEA consent decrees, restriction or suspension of manufacturing and distribution, debarment, refusal to allow product import or export, adverse publicity, refusal of government contracts or future orders under existing contracts, dear-health-care-provider letters or other warnings or corrective information, recalls, delays, significant civil, criminal and administrative penalties including False Claims Act liability, damages, monetary fines, disgorgement, restitution, possible exclusion from participation in Medicare, Medicaid and other federal healthcare programs, corporate integrity agreements, contractual damages, reputational harm, diminished profits and future earnings and curtailment or restructuring of our operations, among other consequences, any of which could adversely affect our ability to operate.

Our current and future relationships with healthcare professionals, principal investigators, consultants, customers and third-party payors in the United States and elsewhere may be subject, directly or indirectly, to applicable anti-kickback, fraud and abuse, false claims, physician payment transparency, and other healthcare laws and regulations, which could expose us to penalties.

Healthcare providers and third-party payors in the United States and elsewhere play a primary role in the recommendation and prescription of our products, as well as any product candidates for which we obtain marketing approval. Our and our commercial partners' current and future arrangements with healthcare professionals, principal investigators, consultants, customers and third-party payors may expose us to broadly applicable fraud and abuse and other healthcare laws, including, without limitation, the Anti-Kickback Statute and the False Claims Act, that may constrain the business or financial arrangements and relationships through which we and our commercial partners sell, market and distribute any product candidates for which we obtain marketing approval. In addition, we may be subject to transparency laws with respect to drug pricing and transfers of value made to physicians and other healthcare professionals. The applicable federal, state and foreign healthcare laws that may affect our ability to operate include the following:

- the federal Anti-Kickback Statute, which prohibits, among other things, persons and entities from knowingly and willfully soliciting, offering, receiving or paying remuneration, directly or indirectly, in cash or in kind, to induce or reward, or in return for, either the referral of an individual for, or the purchase, lease, order or arranging for the purchase, lease or order of, any good, facility, item or service, for which payment may be made, in whole or in part, under federal and state healthcare programs such as Medicare and Medicaid. A person or entity does not need to have actual knowledge of this statute or specific intent to violate it in order to have committed a violation;

- federal civil and criminal false claims laws, including the False Claims Act, which impose criminal and civil penalties, including through civil whistleblower or *qui tam* actions, against individuals or entities for, among other things, knowingly presenting, or causing to be presented, to the federal government, including the Medicare and Medicaid programs, claims for payment that are false or fraudulent or making or using a false record or statement material to a false or fraudulent claim or to avoid, decrease or conceal an obligation to pay money to the federal government, including erroneous pricing information on which mandatory rebates, discounts and reimbursement amounts are based, or in the case of the False Claims Act, for violations of the federal Anti-Kickback Statute in connection with a claim for payment or for conduct constituting reckless disregard for the truth;

- the civil monetary penalties statute, which imposes penalties against any person or entity who, among other things, is determined to have presented or caused to be presented a claim to a federal health program that the person knows or should know is for an item or service that was not provided as claimed or is false or fraudulent;

- the Health Insurance Portability and Accountability Act, or HIPAA, which created additional federal criminal statutes that prohibit knowingly and willfully executing, or attempting to execute, a scheme to defraud any healthcare benefit program or obtain, by means of false or fraudulent pretenses, representations or promises, any of the money or property owned by, or under the custody or control of, any healthcare benefit program, regardless of whether the payor is public or private, knowingly and willfully embezzling or stealing from a health care benefit program, willfully obstructing a criminal investigation of a health care offense and knowingly and willfully falsifying, concealing or covering up by any trick or device a material fact or making any materially false statements in connection with the delivery of, or payment for, healthcare benefits, items or services relating to healthcare matters. Similar to the federal Anti-Kickback Statute, a person or entity does not need to have actual knowledge of this statute or specific intent to violate it in order to have committed a violation;

- the federal Physician Payments Sunshine Act and its implementing regulations, impose new annual reporting requirements for manufacturers of drugs, devices, biologicals and medical supplies for which payment is available under Medicare, Medicaid or the Children's Health Insurance Program, with certain exceptions, to annually report certain payments and transfers of value provided to physicians (defined to include doctors, dentists, optometrists, podiatrists and chiropractors), certain other healthcare professionals (physician assistants, nurse practitioners, clinical nurse specialists, certified nurse anesthetists, anesthesiologist assistants and certified nurse-midwives) and teaching hospitals, or to entities or individuals at the request of, or designated on behalf of, such providers and teaching hospitals, and to report annually certain ownership and investment interests held by physicians and their immediate family members; and

- analogous state and foreign laws and regulations, such as state anti-kickback and false claims laws, which may apply to sales or marketing arrangements and claims involving healthcare items or services reimbursed by non-governmental third-party payors, including private insurers; certain state laws which require drug manufacturers to comply with the industry's voluntary compliance guidelines and the relevant compliance guidance promulgated by the federal government and may require drug manufacturers to report information related to payments and other transfers of value to physicians and other healthcare providers or marketing expenditures; certain state laws that require manufacturers to report information on the pricing of certain drug products; and certain state and local laws require the registration or pharmaceutical sales representatives.

These laws may affect our and our commercial partners' sales, marketing, and other promotional activities by imposing administrative and compliance burdens on us and our commercial partners.

Efforts to ensure that our current and future business arrangements with third parties will comply with applicable healthcare laws and regulations may involve substantial costs. It is possible that governmental authorities will conclude that our business practices may not comply with current or future statutes, regulations or case law involving applicable fraud and abuse or other healthcare laws, or that our compliance systems are inadequate to detect and report such conduct or to report accurate pricing information to the government. If our operations are found to be in violation of any of these laws or any other governmental regulations that may apply to us, we may be subject to significant civil, criminal and administrative penalties, including, without limitation, damages, fines, imprisonment, exclusion from participation in government healthcare programs, such as Medicare and Medicaid, corporate integrity agreements or similar agreements to resolve allegations of non-compliance with these laws, and the curtailment or restructuring of our operations, which could significantly harm our business. If any of the physicians or other healthcare providers or entities with whom we currently, or expect to, do business, including future collaborators, is found not to be in compliance with applicable laws, they and we may be subject to significant penalties and potential exclusion from participation in healthcare programs as a result of their non-compliance.

Recently enacted and future legislation may increase the difficulty and cost for us to obtain marketing approval of our product candidates and increase the cost to commercialize our approved products, and any of our product candidates that may be approved in the future and affect the prices thereof.

In the United States and some foreign jurisdictions, there have been a number of legislative and regulatory changes and proposed changes regarding the healthcare system that could, among other things, prevent or delay marketing approval of our product candidates, restrict or regulate post-approval activities and affect the ability to profitably sell our approved products and our ability to profitably sell any of our product candidates for which we obtain marketing approval.

Among policy makers and payors in the United States and elsewhere, there is significant interest in promoting changes in healthcare systems with the stated goals of containing healthcare costs, improving quality and/or expanding access. In the United States, the pharmaceutical industry has been a particular focus of these efforts and has been significantly affected by major legislative initiatives. For example, in March 2010, the ACA was signed into law and was intended to broaden access to health insurance, reduce or constrain the growth of healthcare spending, enhance remedies against fraud and abuse, add new transparency requirements for the healthcare and health insurance industries, impose new taxes and fees on the health industry and impose additional health policy reforms.

Among the provisions of the ACA of importance to our potential product candidates are the following:

- an annual, nondeductible fee on any entity that manufactures or imports certain branded prescription drugs and biologic agents, apportioned among these entities according to their market share in certain government healthcare programs;
- an increase in the statutory minimum rebates a manufacturer must pay under the Medicaid Drug Rebate Program to 23.1% and 13.0% of the average manufacturer price for branded drugs and generic drugs, respectively;
- expansion of healthcare fraud and abuse laws, including the False Claims Act and the federal Anti-Kickback Statute, new government investigative powers and enhanced penalties for non-compliance;
- establishment of a new and distinct methodology by which rebates owed by manufacturers under the Medicaid Drug Rebate Program are calculated for drugs that are inhaled, infused, instilled, implanted or injected;
- a new Medicare Part D coverage gap discount program, in which manufacturers must agree to offer 70% point-of-sale discounts off negotiated prices (generally as negotiated between the Medicare Part D plan and the pharmacy) of applicable brand drugs to eligible beneficiaries during their coverage gap period, as a condition for the manufacturer's outpatient drugs to be covered under Medicare Part D;
- extension of manufacturers' Medicaid rebate liability to covered drugs dispensed to individuals who are enrolled in Medicaid managed care organizations and extension of the inflation percentage applicable to existing branded drugs to new formulations for purposes of computing the inflation penalty component of Medicaid rebates;
- expansion of eligibility criteria for Medicaid programs by, among other things, allowing states to offer Medicaid coverage to additional individuals and by adding new mandatory eligibility categories for certain individuals with income at or below 133% of the Federal Poverty Level, thereby potentially increasing manufacturers' Medicaid rebate liability;
- expansion of the entities eligible for discounts under the Public Health Service pharmaceutical pricing program;
- the new requirements under the federal Open Payments program and its implementing regulations;
- a new requirement to annually report drug samples that manufacturers and distributors provide to physicians; and
- a new Patient-Centered Outcomes Research Institute to oversee, identify priorities in, and conduct comparative clinical effectiveness research, along with funding for such research.

Since its enactment, there have been executive, judicial and congressional challenges to certain aspects of the ACA. On June 17, 2021, the U.S. Supreme Court dismissed the most recent judicial challenge to the ACA brought by several states without specifically ruling on the constitutionality of the ACA. Thus, the ACA will remain in effect in its current form. Further, prior to the U.S. Supreme Court ruling, President Biden issued an executive order that initiated a special enrollment period from February 15, 2021 through August 15, 2021, for purposes of obtaining health insurance coverage through the ACA marketplace. The executive order also instructed certain governmental agencies to review and reconsider their existing policies and rules that limit access to healthcare, including among others, reexamining Medicaid demonstration projects and waiver programs that include work requirements, and policies that create unnecessary barriers to obtaining access to health insurance coverage through Medicaid or the ACA.

In addition, other legislative changes have been proposed and adopted since the ACA was enacted. In August 2011, the Budget Control Act of 2011, among other things, included aggregate reductions to Medicare payments to providers, which went into effect in April 2013, and, due to subsequent legislative amendments, will stay in effect through 2032, with the exception of a temporary suspension from May 1, 2020 through March 31, 2022 due to the COVID-19 pandemic, unless additional Congressional action is taken. In January 2013, the American Taxpayer Relief Act of 2012 was signed into law, which, among other things, further reduced Medicare payments to several providers, and increased the statute of limitations period for the government to recover overpayments to providers from three to five years. In addition, on March 11, 2021, the American Rescue Plan Act of 2021 was signed into law, which eliminated the statutory Medicaid drug rebate cap, beginning January 1, 2024. The rebate was previously capped at 100% of a drug's average manufacturer price.

Further, there has been increasing legislative and enforcement interest in the United States with respect to specialty drug pricing practices. Specifically, there have been several recent U.S. Congressional inquiries and proposed and enacted federal and state legislation designed to, among other things, bring more transparency to drug pricing, review the relationship between pricing and manufacturer patient programs, reduce the cost of drugs under Medicare, and reform government program reimbursement methodologies for drugs. On August 16, 2022, the Inflation Reduction Act of 2022, or IRA, was signed into law. Among other things, the IRA requires manufacturers of certain drugs to engage in price negotiations with Medicare (beginning in 2026), imposes rebates under Medicare Part B and Medicare Part D to penalize price increases that outpace inflation (first due in 2023), and replaces the Part D coverage gap discount program with a new discounting program (beginning in 2025). The IRA permits the Secretary of the Department of Health and Human Services, or HHS, to implement many of these provisions through guidance, as opposed to regulation, for the initial years. On August 29, 2023, HHS announced the list of the first ten drugs that will be subject to price negotiations, although the drug price negotiation program is currently subject to legal challenges. For that and other reasons, it is currently unclear how the IRA will be effectuated. At the state level, legislatures are increasingly passing legislation and implementing regulations designed to control pharmaceutical and biological product pricing, including price or patient reimbursement constraints, discounts, restrictions on certain product access and marketing cost disclosure and transparency measures, and, in some cases, designed to encourage importation from other countries and bulk purchasing. These new laws may result in additional reductions in Medicare and other healthcare funding, which could negatively impact customers for our product candidates, if approved, and, accordingly, our financial operations.

In the EU, on December 13, 2021, Regulation No 2021/2282 on Health Technology Assessment (“HTA”) amending Directive 2011/24/EU, was adopted. While the Regulation entered into force in January 2022, it will only begin to apply from January 2025 onwards, with preparatory and implementation-related steps to take place in the interim. Once applicable, it will have a phased implementation depending on the concerned products. The Regulation intends to boost cooperation among EU member states in assessing health technologies, including new medicinal products, and provide the basis for cooperation at the EU level for joint clinical assessments in these areas. It will permit EU member states to use common HTA tools, methodologies, and procedures across the EU, working together in four main areas, including joint clinical assessment of the innovative health technologies with the highest potential impact for patients, joint scientific consultations whereby developers can seek advice from HTA authorities, identification of emerging health technologies to identify promising technologies early, and continuing voluntary cooperation in other areas. Individual EU member states will continue to be responsible for assessing non-clinical (e.g., economic, social, ethical) aspects of health technology, and making decisions on pricing and reimbursement. We expect that the healthcare reform measures that have been adopted and may be adopted in the future, may, among other things, result in more rigorous coverage criteria and in additional downward pressure on the price that we receive for any approved product. Any reduction in reimbursement from Medicare or other government programs may result in a similar reduction in payments from private payors. The implementation of cost containment measures or other healthcare reforms may prevent us from being able to generate revenue, attain profitability, or commercialize our prodrug product candidates.

Legislative and regulatory proposals and enacted statutes have been made to expand post-approval requirements and restrict sales and promotional activities for drugs. For instance, the Drug Supply Chain Security Act imposes obligations on manufacturers of pharmaceutical products, among others, related to product tracking and tracing. Among the requirements of this new legislation, manufacturers are required to provide specified information regarding the drug products they produce to individuals and entities to which product ownership is transferred, label drug products with a product identifier and keep specified records regarding the drug products. The transfer of information to subsequent product owners by manufacturers will eventually be required to be done electronically. Manufacturers are also required to verify that purchasers of products are appropriately licensed. Further, under this legislation, manufacturers have drug product investigation, quarantine, disposition and FDA and trading-partner notification responsibilities related to counterfeit, diverted, stolen and intentionally adulterated products, as well as products that are the subject of fraudulent transactions or which are otherwise unfit for distribution such that they would be reasonably likely to result in serious health consequences or death.

In the EU, the EU pharmaceutical legislation is currently undergoing a complete review process, in the context of the Pharmaceutical Strategy for Europe initiative, launched by the European Commission in November 2020. The European Commission’s proposal for revision of several legislative instruments related to medicinal products (potentially reducing the duration of regulatory data protection, revising the eligibility for expedited pathways, etc.) was published on April 26, 2023. The proposed revisions remain to be agreed and adopted by the European Parliament and European Council and the proposals may therefore be substantially revised before adoption, which is not anticipated before early 2026. The revisions may however have a significant impact on the pharmaceutical industry and our business in the long term.

We cannot be sure whether additional legislative changes will be enacted, or whether the FDA or foreign regulations, guidance or interpretations will be changed, or what the impact of such changes on the marketing approvals of our product candidates, if any, may be. In addition, increased scrutiny by the U.S. Congress of the FDA's approval process may significantly delay or prevent marketing approval, as well as subject us to more stringent product labeling and post-marketing testing and other requirements.

Governments outside the United States tend to impose strict price controls, which may affect our revenue, if any.

In some countries, particularly the EU member states, the pricing of prescription pharmaceuticals is subject to governmental control. In these countries, pricing negotiations with governmental authorities can take considerable time after the receipt of marketing approval for a product. To obtain coverage and reimbursement or pricing approval in some member states, we may be required to conduct a clinical trial that compares the cost-effectiveness of our product candidate to other available therapies. If reimbursement of our prodrug products is unavailable or limited in scope or amount, or if pricing is set at unsatisfactory levels, our business could be harmed, possibly materially.

If we fail to comply with environmental, health and safety laws and regulations, we could become subject to fines or penalties or incur costs that could harm our business.

We are subject to numerous environmental, health and safety laws and regulations, including those governing laboratory procedures and the handling, use, storage, treatment and disposal of hazardous materials and wastes. Our operations involve the use of hazardous and flammable materials, including chemicals and biological materials. Our operations also produce hazardous waste products. We generally contract with third parties for the disposal of these materials and wastes. We cannot eliminate the risk of contamination or injury from these materials. In the event of contamination or injury resulting from our use of hazardous materials, we could be held liable for any resulting damages, and any liability could exceed our resources. We also could incur significant costs associated with civil or criminal fines and penalties for failure to comply with such laws and regulations.

Although we maintain workers' compensation insurance to cover us for costs and expenses we may incur due to injuries to our employees resulting from the use of hazardous materials, this insurance may not provide adequate coverage against potential liabilities. We do not maintain insurance for environmental liability or toxic tort claims that may be asserted against us in connection with our storage or disposal of biological, hazardous or radioactive materials.

In addition, we may incur substantial costs in order to comply with current or future environmental, health and safety laws and regulations. These current or future laws and regulations may impair our research, development or production efforts. Our failure to comply with these laws and regulations also may result in substantial fines, penalties or other sanctions.

If our security measures are compromised now, or in the future, or the security, confidentiality, integrity or availability of, our information technology systems, software, services, communications or data is compromised, limited or fails, this could result in a material adverse impact, including without limitation, a material interruption to our operations, harm to our reputation, significant fines, penalties and liability, breach or a triggering of data protection laws, privacy policies and data protection obligations, loss of customers or sales, or material disruption of our clinical trials or other business activity.

In the ordinary course of our business, we may collect, process and store proprietary, confidential and sensitive information, including personal information (including key-coded data and health information), intellectual property, trade secrets and proprietary business information owned or controlled by ourselves or other parties.

There can be no assurance that our cybersecurity risk management program and processes, including our policies, controls or procedures, will be fully implemented, complied with or effective in protecting our systems and information. Despite the implementation of security measures, our information technology systems and data, and those of our CROs and other third parties on which we rely, are vulnerable to system failure, interruption, compromise, attack or damage from several sources, such as data corruption; breakdown; malicious human acts; malware (such as ransomware); malicious code (such as computer viruses or worms); fraudulent activity; employee misconduct, theft or error; denial-of-service attacks; public health epidemics; cyber-attacks by sophisticated nation-state and nation-state supported actors; natural disasters; terrorism; war (such as the current situation with Ukraine and Russia); and telecommunication and electrical failures. Our recovery systems (and those of third parties upon whom we rely) are similarly vulnerable. Any of these events could lead to the unauthorized access, disclosure and use of proprietary, confidential, or otherwise non-public information (such as personal information). The techniques used by criminal actors to attack computer systems are sophisticated, change frequently and may originate from less regulated and remote areas of the world. As a result, we may not be able to address these techniques proactively or implement adequate preventative measures. Attacks upon information technology systems are also increasing in their levels of persistence and intensity and are being conducted by sophisticated and organized groups and individuals with a wide range of motives and expertise. As a result of the continued hybrid working environment, we may also face increased cybersecurity risks due to our reliance on internet technology and the number of our employees who are working remotely, which may create additional opportunities for cybercriminals to exploit vulnerabilities. We may also experience security breaches that may remain undetected for an extended period. We may be required to expend significant resources, fundamentally change our business activities and practices, or modify our operations, including our clinical trial activities, or information technology in an effort to protect against security breaches and to mitigate, detect and remediate actual and potential vulnerabilities. Due to the nature of some of these threats, we may be unable to anticipate threats, and there is a risk that a threat may remain undetected for a period of time. The costs of maintaining or upgrading our cyber-security systems at the level commercially reasonable to keep up with our expanding operations and prevent against potential attacks are increasing, and despite our best efforts, our network security and data recovery measures and those of our vendors may still not be adequate to protect against such security breaches and disruptions, which could cause harm to our business, financial condition and results of operations.

We and certain of our service providers are from time to time subject to cyberattacks and security incidents. While we do not believe that we have experienced any significant system failure, accident or security breach to date, if we, our service providers, partners, or other relevant third parties experience any such incident that results in any data loss, deletion or destruction, unauthorized access to, loss of, acquisition or disclosure of, exposure or disclosure of sensitive information, or compromise related to the security, confidentiality, integrity or availability of our (or their) information technology, software, services, communications or data, it may result in a material adverse impact, including without limitation, fines, damages, litigation, enforcement actions, loss of trade secrets, a material disruption of our drug development programs, or other harm to our business. For example, the loss of clinical trial data from completed or ongoing or planned clinical trials could result in delays in our regulatory approval efforts and significantly increase our costs to recover or reproduce the data. To the extent that any disruption or security breach was to result in a loss of or damage to our data or applications, or inappropriate disclosure of confidential or proprietary information, we could incur liability and the further development of our product candidates could be delayed. Additionally, applicable data protection laws, privacy policies and data protection obligations (such as contractual obligations) may require us to notify relevant stakeholders of security breaches, including affected individuals, customers and regulators. Such disclosures are costly, and the disclosure or the failure to comply with such requirements, could lead to material adverse impacts, including without limitation, negative publicity, a loss of confidence in our products or operations or breach of contract claims. There can be no assurance that the limitations of liability in our contracts would be enforceable or adequate or would otherwise protect us from liabilities or damages if we fail to comply with applicable data protection laws, privacy policies or data protection obligations related to information security or security breaches.

Further, failures or significant downtime of our information technology or telecommunication systems or those used by our third-party service providers could cause significant interruptions in our operations and adversely impact the confidentiality, integrity and availability of sensitive or confidential information, including preventing us from conducting clinical trials, tests or research and development activities and preventing us from managing the administrative aspects of our business.

We cannot be sure that our insurance coverage will be adequate or otherwise protect us from or adequately mitigate liabilities or damages with respect to claims, costs, expenses, litigation, fines, penalties, business loss, data loss, regulatory actions or materially adverse impacts arising out of our privacy and cybersecurity practices, processing or security breaches that we may experience, or that such coverage will continue to be available on acceptable terms at all. The successful assertion of one or more large claims against us that exceeds our available insurance coverage, or results in changes to our insurance policies (including premium increases or the imposition of a large deductible or co-insurance requirements), could have an adverse effect on our business. In addition, we cannot be sure that our existing coverage and coverage for errors and omissions will continue to be available on acceptable terms or that our insurers will not deny coverage as to any future claim.

Failure or perceived failure to comply with existing or future laws, regulations, contracts, self-regulatory schemes, policies, standards and other obligations related to data privacy or security could lead to government enforcement actions (which could include civil or criminal fines or penalties), a disruption of our clinical trials or commercialization of our products, private litigation, other liabilities, and/or adverse publicity. Compliance or the failure to comply with such obligations could increase the costs of our products, could limit their use or adoption, and could otherwise negatively affect our operating results and business.

Regulation of data (including personal data) is evolving, as federal, state, and foreign governments continue to adopt new, or modify existing, laws and regulations addressing data privacy and security, and the collection, processing, storage, transfer, and use of data. We and our collaborators may be subject to current, new, or modified federal, state, and foreign data protection laws and regulations. These new or proposed laws and regulations are subject to differing interpretations and may be inconsistent among jurisdictions, and guidance on implementation and compliance practices are often updated or otherwise revised, which adds to the complexity of processing personal data. Moreover, we are subject to the terms of our privacy and security policies, representations, certifications, standards, publications, contracts and other obligations to third parties related to data privacy, security and processing. These and other requirements could require us or our collaborators to incur additional costs to achieve compliance, limit our competitiveness, necessitate the acceptance of more onerous obligations in our contracts, restrict our ability to use, store, transfer, and process data, impact our or our collaborators' ability to process or use data in order to support the provision of our products, affect our or our partners' ability to offer our products or operate in certain locations, cause regulators to reject, limit, or disrupt our clinical trial activities, result in increased expenses, reduce overall demand for our products and services and make it more difficult to meet expectations of or commitments to customers or collaborators.

Furthermore, the Federal Trade Commission, or the FTC, also has authority to initiate enforcement actions against entities that mislead customers about HIPAA compliance, make deceptive statements about privacy and data sharing in privacy policies, fail to limit third-party use of personal health information, fail to implement policies to protect personal health information or engage in other unfair practices that harm customers or that may violate Section 5 of the FTC Act. Even when HIPAA does not apply, failing to take appropriate steps to keep consumers' personal information secure can constitute unfair acts or practices in or affecting commerce in violation of Section 5(a) of the FTC Act. The FTC expects a company's data security measures to be reasonable and appropriate in light of the sensitivity and volume of consumer information it holds, the size and complexity of its business, and the cost of available tools to improve security and reduce vulnerabilities. Additionally, federal and state consumer protection laws are increasingly being applied by FTC and states' attorneys general to regulate the collection, use, storage, and disclosure of personal or personally identifiable information, through websites or otherwise, and to regulate the presentation of website content.

Certain states have also adopted comparable privacy and security laws and regulations, some of which may be more stringent than HIPAA. For example, the California Consumer Privacy Act of 2018, as amended by the California Privacy Rights Act, collectively the CCPA, requires businesses that process the personal information of the California residents to, among other things, (i) provide certain disclosures to California residents regarding the business's collection, use, and disclosure of their personal information (ii) receive and respond to requests from California residents to access, delete, and correct their personal information or opt out of certain disclosures of their personal information; and (iii) enter into specific contractual provisions with service providers that process California resident personal information on the business's behalf. Similar laws have been passed in other states and are continuing to be proposed at the state and federal level, reflecting a trend toward more stringent privacy legislation in the United States. As we expand our operations, these and similar laws may increase our compliance costs and potential liability

Foreign data protection laws, such as the GDPR and member state data protection laws, may also apply to health-related and other personal data that we process, including personal data relating to clinical trial participants. European data protection laws impose strict obligations on the ability to process health-related and other personal data of data subjects in Europe, including standards relating to the privacy and security of personal data. For example, the GDPR went into effect in May 2018 and imposes strict requirements for processing the personal data of individuals within the EEA. Companies that must comply with the GDPR face increased compliance obligations and risk, including more robust regulatory enforcement of data protection requirements and potential fines for noncompliance of up to €20 million or 4% of the annual global revenues of the noncompliant company, whichever is greater. Among other requirements, the GDPR regulates transfers of personal data subject to the GDPR to third countries that have not been found to provide adequate protection to such personal data, including the United States, and the efficacy and longevity of current transfer mechanisms between the EEA and the United States remains uncertain. Case law from the Court of Justice of the European Union, or CJEU, states that reliance on the standard contractual clauses - a standard form of contract approved by the European Commission as an adequate personal data transfer mechanism - alone may not necessarily be sufficient in all circumstances and that transfers must be assessed on a case-by-case basis. On October 7, 2022, President Biden signed an Executive Order on 'Enhancing Safeguards for United States Intelligence Activities' which introduced new redress mechanisms and binding safeguards to address the concerns raised by the CJEU in relation to data transfers from the EEA to the United States and which formed the basis of the new EU-US Data Privacy Framework, or DPF, as released on December 13, 2022. The European Commission adopted its Adequacy Decision in relation to the DPF on July 10, 2023, rendering the DPF effective as a GDPR transfer mechanism to U.S. entities self-certified under the DPF. The DPF also introduced a new redress mechanism for EU citizens which addresses a key concern in the previous CJEU judgments and may mean transfers under standard contractual clauses are less likely to be challenged in future. We currently rely on [the EU standard contractual clauses] [and] [the UK Addendum to the EU standard contractual clauses and the UK International Data Transfer Agreement and the DPF as relevant to transfer personal data outside the EEA and the UK, including to the United States, with respect to both intragroup and third-party transfers. We expect the existing legal complexity and uncertainty regarding international personal data transfers to continue. In particular, we expect the DPF Adequacy Decision to be challenged and international transfers to the United States and to other jurisdictions more generally to continue to be subject to enhanced scrutiny by regulators. As a result, we may have to make certain operational changes and we will have to implement revised standard contractual clauses and other relevant documentation for existing data transfers within required time frames As supervisory authorities issue further guidance on personal data export mechanisms, including circumstances where the standard contractual clauses cannot be used, and/or start taking enforcement action, we could suffer additional costs, complaints and/or regulatory investigations or fines, and/or if we are otherwise unable to transfer personal data between and among countries and regions in which we operate, it could affect the manner in which we provide our services, the geographical location or segregation of our relevant systems and operations, and could adversely affect our financial results. Additionally, other countries have passed or are considering passing laws requiring local data residency and/or restricting the international transfer of data.

Further, from January 1, 2021, companies have had to comply with the GDPR and also the UK data protection regime, which imposes separate but similar obligations to those under the GDPR in UK national law. The UK GDPR mirrors the fines under the GDPR, i.e., fines up to the greater of €20 million (£17.5 million) or 4% of global turnover. On October 12, 2023, the UK Extension to the DPF came into effect (as approved by the UK government), as a UK GDPR data transfer mechanism to U.S. entities self-certified under the UK Extension to the DFP. As we continue to expand into other foreign countries and jurisdictions, we may be subject to additional laws and regulations that may affect how we conduct business.

Failure, or perceived failure, to comply with federal, state and foreign data protection laws and regulations, privacy policies, contracts and other data protection obligations could result in government investigations and enforcement actions (which could include civil or criminal penalties, fines, or sanctions), private litigation, a diversion of management's attention, adverse publicity and other negative effects on our operating results and business. There can be no assurance that the limitations of liability in our contracts would be enforceable or adequate or would otherwise protect us from liabilities or damages if we fail to comply with applicable data protection laws, privacy policies or data protection obligations related to information security or security breaches. Moreover, clinical trial participants or subjects about whom we or our collaborators obtain information, as well as the providers who share this information with us, may contractually limit our ability to use and disclose the information. Claims that we have violated individuals' privacy rights or failed to comply with data protection laws, contracts, privacy notices or other obligations even if we are not found liable, could be expensive and time-consuming to defend and could result in adverse publicity that could harm our business.

Any of these matters could materially adversely affect our business, financial condition, or operational results.

Risks Related to Employee Matters and Managing Our Growth

Our future success depends on our ability to retain key executives and to attract, retain and motivate qualified personnel.

We are highly dependent on the management, research and development, clinical, financial and business development expertise of our senior leadership team, as well as the other members of our scientific and clinical teams. Although we have employment agreements with each of our executive officers, these agreements do not obligate them to continue working for our company and they may terminate their employment with us at any time. Among other recent changes in our senior management team, our new Chief Executive Officer was appointed effective October 10, 2023. Our future performance will depend, in part, on a successful transition period with our new Chief Executive Officer, Neil F. McFarlane, the successful integration of any other new senior level executives into their roles, and the continuity of leadership among the larger workforce. If we do not successfully manage these transitions, it could be viewed negatively by our customers, employees, investors, and other third-party partners, and could have an adverse impact on our business and results of operations.

Recruiting and retaining qualified scientific and clinical personnel and, if we progress the development of our product candidate pipeline toward scaling up for commercialization, manufacturing and sales and marketing personnel, will also be critical to our success. The loss of the services of our executive officers or other key employees could impede the achievement of our research, development and commercialization objectives and seriously harm our ability to successfully implement our business strategy. Furthermore, replacing executive officers and key employees may be difficult and may take an extended period of time because of the limited number of individuals in our industry with the breadth of skills and experience required to successfully develop, gain regulatory approval of and commercialize our prodrug product candidates. Competition to hire from this limited pool is intense, and we may be unable to hire, train, retain or motivate these key personnel on acceptable terms given the competition among numerous pharmaceutical and biotechnology companies for similar personnel. We also experience competition for the hiring of scientific and clinical personnel from universities and research institutions. In addition, we rely on consultants and advisors, including scientific and clinical advisors, to assist us in formulating our research and development and commercialization strategy. Our consultants and advisors may have commitments under consulting or advisory contracts with other entities that may limit their availability to us. If we are unable to continue to attract and retain high quality personnel, our ability to pursue our growth strategy will be limited.

Risks Related to Ownership of Our Common Stock and Our Status as a Public Company

The trading price of the shares of our common stock is likely to be volatile, and purchasers of our common stock could incur substantial losses.

Our stock price has been, and is likely to continue to be, volatile. Since shares of our common stock were sold in our initial public offering in April 2015 at a price of $176.00 per share (adjusted to give effect to the 1-for-16 reverse stock split), our stock price has ranged from a low of $1.94 to a high of $418.40 through April 1, 2024. In addition, the stock market in general and the market for pharmaceutical companies in particular have experienced extreme volatility that has often been unrelated to the operating performance of particular companies. As a result of this volatility, investors may not be able to sell their common stock at or above the price paid for the shares. The market price for our common stock may be influenced by many factors, including:

- actual or anticipated variations in our operating results;
- changes in financial estimates by us or by any securities analysts who might cover our stock;
- conditions or trends in our industry, including without limitation changes in the structure of healthcare payment systems;
- stock market price and volume fluctuations of comparable companies and, in particular, those that operate in the pharmaceutical industry;
- announcements by us or our competitors of significant acquisitions, strategic partnerships or divestitures;
- announcements of investigations or regulatory scrutiny of our operations or lawsuits filed against us;
- adverse regulatory announcements or determinations regarding our product candidates;
- capital commitments;
- investors' general perception of us and our business;
- global macroeconomic conditions, including inflation, labor shortages, supply chain shortages, or other economic, political or legal uncertainties or adverse developments;
- political unrest, terrorism and wars, such as the current situation with Ukraine and Russia or Israel and Hamas, which could delay or disrupt our business, and if such political unrest escalates or spills over to or otherwise impacts additional regions it could heighten many of the other risk factors included in this Item 1A;
- other events or factors, including those resulting from system failures and disruptions, earthquakes, hurricanes, other natural disasters, pandemics, or responses to these events;
- recruitment or departure of key personnel; and
- sales of our common stock, including sales by our directors and officers or specific stockholders.

Many of the factors described above are not within our control and we cannot guarantee that future instances of these influencing factors will not have effects on the trading price of our common stock.

In addition, in the past, stockholders have initiated class action lawsuits against pharmaceutical and biotechnology companies following periods of volatility in the market prices of these companies' stock. For instance, in December 2016, we received notice of a class action suit filed against us in the Iowa District Court in Johnson County by a stockholder alleging that we, certain of our senior executives and directors who signed the registration statement in connection with our initial public offering, and each of the investment banks that acted as underwriters for the offering negligently issued untrue statements of material facts and omitted to state material facts required to be stated in the registration statement and incorporated offering materials that we filed with the U.S. Securities and Exchange Commission, or the SEC, in support of the offering. In June 2018, the case was dismissed without prejudice to members of the putative class. Future litigation could cause us to incur substantial costs and divert management's attention and resources from our business. Further, biotechnology and pharmaceutical companies in particular, have experienced extreme price and volume fluctuations that have often been unrelated or disproportionate to the operating performance of these companies. Broad market and industry factors may negatively affect the market price of our common stock, regardless of our actual operating performance.

A portion of our outstanding warrants are entitled to certain anti-dilution protections which, if triggered, may cause dilution to your investment.

The warrant held by OTA LLC, or the OTA Warrant, includes an exercise price protection provision, pursuant to which the exercise price of the OTA Warrant will be adjusted downward on a broad-based weighted-average basis if we issue or sell any shares of common stock, convertible securities, warrants or options at a sale or exercise price per share less than the greater of (i) $38.34 per share, which represents the OTA Warrant's exercise price, or (ii) the closing sale price of our common stock on the last trading date immediately prior to such issuance or, in the case of a firm commitment underwritten offering, on the date of execution of the underwriting agreement between us and the underwriters for such offering. Additionally, if we effect an "at the market offering", as defined in Rule 415 of the Securities Act, of our common stock, the exercise price of the OTA Warrant will be adjusted downward pursuant to this anti-dilution adjustment only if such sales are made at a price less than $38.34 per share, provided that this anti-dilution adjustment will not apply to certain specified sales.

Future sales and issuances of equity and debt could result in additional dilution to our stockholders.

We may need additional capital in the future to fund our planned future operations, including to complete potential clinical trials for our product candidates or for successful commercialization of our approved products. To raise capital, we may sell or issue common stock, convertible securities or other equity securities in one or more transactions at prices and in a manner we determine from time to time. The issuance of additional shares of our common stock or equity securities convertible into shares of our common stock may cause your ownership interest to be diluted, and the market price of our common stock may fall or be materially and adversely effected. Examples of such issuances of equity securities include the following:

- The holders of any of the warrants described elsewhere in this Annual Report on Form 10-K may elect to exercise such warrants and receive shares of common stock.
- In connection with the Merger and its related transactions, we issued shares of our common stock pursuant to the Loan and Note Purchase Agreements and the cancellation of the Acer warrants. See "Liquidity and Capital Resources."
- In July 2021, the Company entered into the Equity Distribution Agreement with JMP Securities LLC, or JMP, and RBC Capital Markets, LLC, or RBCCM, under which the Company may offer and sell, from time to time at its sole discretion, shares of its common stock having an aggregate offering price of up to $75,000,000 through JMP and RBCCM as its sales agents.

In June 2021, at the annual meeting of stockholders, the stockholders approved the Amended and Restated 2014 Equity Incentive Plan, which, among other things, added 4,900,000 shares to the equity plan pool, and the employee stock purchase plan, or the 2021 ESPP, which allows for employees to purchase up to 1,500,000 million shares of stock through the plan. Pursuant to our equity incentive plan, we may grant equity awards and issue additional shares of our common stock to our employees, directors and consultants, and the number of shares of our common stock reserved for future issuance under this plan will be subject to automatic annual increases in accordance with the terms of the plans.

In February 2024, our Board approved the Amended and Restated 2023 Employment Inducement Award Plan, (the "2023 Plan"), under which we may grant equity awards to certain new employees. The maximum number of shares of common stock to be issued under the 2023 Plan is 4,500,000.

Additionally, in February 2024, our Board approved amendments to the Amended and Restated 2014 Equity Incentive Plan, (the "2014 Plan"), under which we may grant equity awards to certain new employees. The maximum number of shares of common stock to be issued under the 2014 Plan, if amended, will be 19,600,000. The amendments to the 2014 plan are subject to stockholder approval at our 2024 annual meeting of stockholders.

Sales of a substantial number of shares of our common stock in the public market could cause the market price of our common stock to drop significantly, even if our business is doing well.

Sales of a substantial number of shares of our common stock in the public market could occur at any time. If our stockholders sell, or the market perceives that our stockholders intend to sell, substantial amounts of our common stock in the public market, the market price of our common stock could decline significantly.

On December 20, 2021, we initiated a share repurchase program, or the Share Repurchase Program, pursuant to which we may repurchase up to $50 million of shares of our common stock through December 31, 2023. On December 31, 2023, the Share Repurchase Program ended, and we had repurchased 1,575,692 shares of our common stock for approximately $11.0 million.

Anti-takeover provisions in our amended and restated certificate of incorporation and amended and restated bylaws, as well as provisions of Delaware law and the terms of some or our contracts, might discourage, delay or prevent a change in control of our company or changes in our board of directors or management and, therefore, depress the price of our common stock.

Our amended and restated certificate of incorporation and amended and restated bylaws and Delaware law contain provisions that may discourage, delay or prevent a merger, acquisition or other change in control that stockholders may consider favorable, including transactions in which you might otherwise receive a premium for your shares of our common stock or transactions that our stockholders might otherwise deem to be in their best interests. These provisions may also prevent or frustrate attempts by our stockholders to replace or remove members of our board of directors or our management. Therefore, these provisions could adversely affect the price of our stock. Our corporate governance documents include provisions:

- establishing a classified board of directors with staggered three-year terms so that not all members of our board of directors are elected at one time;
- providing that directors may be removed by stockholders only for cause;
- preventing the ability of our stockholders to call and bring business before special meetings and to take action by written consent in lieu of a meeting;
- requiring advance notice of stockholder proposals for business to be conducted at meetings of our stockholders and for nominations of candidates for election to our board of directors;
- permitting the board of directors to issue up to 10,000,000 shares of preferred stock with any rights, preferences and privileges they may designate;
- limiting the liability of, and providing indemnification to, our directors and officers;
- providing that vacancies may be filled by remaining directors;
- preventing cumulative voting; and
- providing for a supermajority requirement to amend our amended and restated bylaws.

As a Delaware corporation, we are also subject to provisions of Delaware law, including Section 203 of the General Corporation Law of the State of Delaware, which prohibits a Delaware corporation from engaging in a broad range of business combinations with any "interested" stockholder for a period of three years following the date on which the stockholder became an "interested" stockholder.

In addition, the provisions of our termination agreement with Aquestive may delay or prevent a change in control of our company. For example, if we enter into a merger, an asset sale or any other change of control transaction, then Aquestive will be entitled to a royalty equal to 10% of the price being paid to us and our stockholders in such transaction which is attributable to the value of AZSTARYS or KP1077.

Any provision of our amended and restated certificate of incorporation, amended and restated bylaws or Delaware law or any term of our contracts that has the effect of discouraging, delaying or preventing a change in control could limit the opportunity for our stockholders to receive a premium for their shares of our common stock and could also affect the price that some investors are willing to pay for our common stock.

If we engage in acquisitions to grow our business, we will incur a variety of costs and may potentially face numerous risks that could adversely affect our business and operations and cause our stock price to decline.

If appropriate opportunities become available, we may seek to acquire businesses or assets to enhance our business. In connection with any acquisitions, we could issue additional equity securities, which would dilute our stockholders, incur substantial debt to fund the acquisitions or assume significant liabilities.

Acquisitions involve many and diverse risks and uncertainties, including problems integrating the purchased operations or assets as well as unanticipated costs, liabilities, and economic, political, legal and regulatory challenges due to our inexperience operating in new regions or countries, and we may fail to successfully integrate acquired companies, such as Orphazyme, or retain key personnel from the acquired company. To date, we have limited experience with acquisitions and the integration of acquired operations and personnel. Acquisitions may divert our attention from our core business. Acquisitions may require us to record goodwill and non-amortizable intangible assets that will be subject to testing on a regular basis and potential period impairment charges, incur amortization expenses related to certain intangible assets, and incur write offs and restructuring and other related expenses, any of which could harm our operating results and financial condition.

New business strategies, especially those involving acquisitions, are inherently risky and may not be successful. Failure to successfully identify, complete, manage and integrate acquisitions could materially and adversely affect our business, financial condition and results of operations and could cause our stock price to decline.

We could be negatively affected as a result of the actions of activist stockholders, which could be disruptive and costly and may conflict with or disrupt the strategic direction of our business.

In January 2023, our board of directors received notice from a stockholder of his intention to nominate three nominees to stand for election to our board of directors at our 2023 annual meeting of stockholders and to submit a proposal at the annual meeting, which resulted in a contested election at the annual meeting at which such nominees were elected by our stockholders. Similar to the activist stockholder activities initiated in January 2023, activist stockholders may from time to time attempt to effect changes in our strategic direction and seek changes regarding our corporate governance or structure. Our board of directors and management team strive to maintain constructive, ongoing communications with all stockholders who wish to speak with us, including activist stockholders, and welcome their views and opinions with the goal of working together constructively to enhance value for all stockholders. Any future proxy contest with respect to election of our directors, or other activist stockholder activities, could adversely affect our business because: (1) responding to a proxy contest and other actions by activist stockholders can be costly and time-consuming, disruptive to our operations and divert the attention of management and our employees; (2) actual or perceived uncertainties as to our future direction caused by activist activities may cause or appear to cause instability or lack of continuity, resulting in the loss of potential business opportunities, and potentially making it more difficult to attract and retain qualified personnel and business partners; and (3) if individuals are elected to our board of directors with a specific agenda, it may adversely affect our ability to effectively and timely implement our strategic plans. Activist stockholder activities may also cause significant fluctuations in our stock price based on temporary or speculative market perceptions, or other factors that do not necessarily reflect the fundamental underlying value of our business.

We may be subject to securities litigation, class action and derivative lawsuits, which could result in substantial costs and could divert management attention away from other business concerns.

Securities class action lawsuits and derivative lawsuits are often brought against public companies that have entered into merger agreements. Even if the lawsuits are without merit, defending against these claims can result in substantial costs and divert management time and resources from other business concerns, which could seriously harm our business. An adverse judgment could result in monetary damages, which could have a negative impact on our liquidity and financial condition. For example, in October 2023, purported stockholders of Acer filed complaints against Acer and certain of its directors and officers in connection with the Merger. See Note H of our audited consolidated financial statements included elsewhere in this Annual Report on Form 10-K. The Company does not believe the allegations in the complaints and demand letters are meritorious, and intends to defend against them vigorously.

Securities litigation against us could result in substantial costs and divert management's attention from other business concerns, which could seriously harm our business.

Our amended and restated certificate of incorporation provides that the Court of Chancery of the State of Delaware is the exclusive forum for substantially all disputes between us and our stockholders, which could limit our stockholders' ability to obtain a favorable judicial forum for disputes with us or our directors, officers or other employees.

Our amended and restated certificate of incorporation provides that the Court of Chancery of the State of Delaware is the sole and exclusive forum for (i) any derivative action or proceeding brought on our behalf, (ii) any action asserting a breach of fiduciary duty owed by any of our directors, officers or other employees to us or our stockholders, (iii) any action asserting a claim against us arising pursuant to any provisions of the Delaware General Corporation Law, or DGCL, our amended and restated certificate of incorporation or our amended and restated bylaws, or (iv) any action asserting a claim against us that is governed by the internal affairs doctrine.

In addition, on and effective July 15, 2020, we amended and restated our amended and restated bylaws, or the Bylaws, pursuant to which: (i) unless we consent in writing to the selection of an alternative forum, the Court of Chancery of the State of Delaware (or, if and only if the Court of Chancery of the State of Delaware lacks subject matter jurisdiction, any state court located within the State of Delaware or, if and only if all such state courts lack subject matter jurisdiction, the federal district court for the District of Delaware) shall be the sole and exclusive forum for the following types of actions or proceedings under Delaware statutory or common law: (A) any derivative action or proceeding brought on behalf of us; (B) any action or proceeding asserting a claim of breach of a fiduciary duty owed by any of our current or former directors, officers or other employees, to us or our stockholders; (C) any action or proceeding asserting a claim against us or any of our current or former directors, officers or other employees, arising out of or pursuant to any provision of the DGCL our amended and restated certificate of incorporation or our Bylaws (as each may be amended from time to time); (D) any action or proceeding to interpret, apply, enforce or determine

the validity of our amended and restated certificate of incorporation or our Bylaws (including any right, obligation, or remedy thereunder); (E) any action or proceeding as to which the DGCL confers jurisdiction to the Court of Chancery of the State of Delaware; and (F) any action or proceeding asserting a claim against us or any of our directors, officers or other employees, governed by the internal affairs doctrine, in all cases to the fullest extent permitted by law and subject to the court's having personal jurisdiction over the indispensable parties named as defendants, *provided that* this provision shall not apply to suits brought to enforce a duty or liability created by the Securities Act or the Exchange Act, or any other claim for which the federal courts have exclusive jurisdiction; (ii) unless we consent in writing to the selection of an alternative forum, to the fullest extent permitted by law, the federal district courts of the United States of America shall be the exclusive forum for the resolution of any complaint asserting a cause of action arising under the Securities Act; and (iii) any person or entity holding, owning or otherwise acquiring any interest in any security of us shall be deemed to have notice of and consented to the provisions of the Bylaws.

These choice of forum provision may limit a stockholder's ability to bring a claim in a judicial forum that it finds favorable for disputes with us or our directors, officers or other employees, which may discourage such lawsuits against us and our directors, officers and other employees. If a court were to find the choice of forum provision contained in our amended and restated certificate of incorporation or our Bylaws to be inapplicable or unenforceable in an action, we may incur additional costs associated with resolving such action in other jurisdictions.

We might not be able to utilize a significant portion of our net operating loss carryforwards, which could adversely affect our profitability.

As of December 31, 2023, we had federal net operating loss carryforwards of approximately $350 million, due to prior period losses, $145.3 million of which, if not utilized, will begin to expire in 2027 and $204.7 million of which have no expiration date. These net operating loss carryforwards could expire unused and be unavailable to offset future income tax liabilities, which could adversely affect our profitability. These federal net operating loss carryforwards are fully reserved under a valuation allowance in the consolidated balance sheet as of December 31, 2023. On December 22, 2017, the U.S. government enacted H.R. 1, "An Act to provide for reconciliation pursuant to titles II and V of the concurrent resolution on the budget for fiscal year 2018" (informally titled the Tax Cuts and Jobs Act). Under the Tax Cuts and Jobs Act, U.S. federal net operating losses incurred in 2018 and in future years may be carried forward indefinitely, but the deductibility of such federal net operating losses is limited. It is uncertain if and to what extent various states will conform to the Tax Cuts and Jobs Act. To the extent that we continue to generate taxable losses in the United States, unused losses will carry forward to offset future taxable income (subject to any applicable limitations), if any. In addition, under Section 382 and Section 383 of the Code, if a corporation undergoes an ''ownership change,'' which is generally defined as a greater than 50% change, by value, in its equity ownership over a three-year period, the corporation's ability to use its pre-change net operating loss carryforwards and other pre-change tax attributes to offset its post-change income may be limited. We performed a Section 382 ownership change analysis in 2017 and determined that we experienced an ownership change in 2010, which resulted in a portion of our net operating loss carryforwards being subject to an annual limitation under Section 382 through 2012. No other ownership changes or limitations on our historical net operating loss carryforwards were noted through the year ended December 31, 2017. In addition, we may have already experienced an ownership change, or may experience ownership changes in the future, as a result of shifts in our stock ownership, including as a result of the Merger, as a result of the conversion of our outstanding convertible debt or otherwise as a result of changes in our stock ownership. If we determine that an ownership change has occurred and our ability to use our historical net operating loss carryforwards is materially limited, it would harm our future operating results by increasing our future tax obligations. As of December 31, 2023, we maintain a full valuation allowance over our deferred tax assets for financial reporting purposes.

Changes in tax laws or regulations that are applied adversely to us may have a material adverse effect on our business, cash flow, financial condition or results of operations.

New income, sales, use or other tax laws, statutes, rules, regulations or ordinances could be enacted at any time, which could affect the tax treatment of our domestic earnings, if any. Any new taxes could adversely affect our business operations, and our business and financial performance. Further, existing tax laws, statutes, rules, regulations or ordinances could be interpreted, changed, modified or applied adversely to us. For example, legislation enacted in 2017, informally titled the Tax Cuts and Jobs Act, significantly revised the Internal Revenue Code of 1986, as amended. Future guidance from the Internal Revenue Service and other tax authorities with respect to the Tax Cuts and Jobs Act may affect us, and certain aspects of the Tax Cuts and Jobs Act could be repealed or modified in future legislation. In addition, the Inflation Reduction Act enacted in 2022 in the United States introduced, among other changes, a 15% corporate minimum tax on certain United States corporations and a 1% excise tax on certain stock redemptions by United States corporations. Furthermore, it is uncertain if and to what extent various states will conform to the Tax Cuts and Jobs Act or any newly enacted federal tax legislation. Changes in corporate tax rates, the realization of net deferred tax assets relating to our operations, the taxation of foreign earnings, and the deductibility of expenses under the Tax Cuts and Jobs Act or future reform legislation could have a material impact on the value of our deferred tax assets, could result in significant one-time charges, and could increase our future U.S. tax expense.

If we fail to maintain proper and effective internal control, our ability to produce accurate financial statements on a timely basis could be impaired.

We are subject to the reporting requirements of the Exchange Act, the Sarbanes-Oxley Act of 2002, or the Sarbanes-Oxley Act, and the rules and regulations of the stock market on which our common stock is listed. The Sarbanes-Oxley Act requires, among other things, that we maintain effective disclosure controls and procedures and internal control over financial reporting. For our fiscal year ended December 31, 2023, we performed system and process evaluation and testing of our internal control over financial reporting to allow management to report on the effectiveness of our internal control over financial reporting in our Annual Report on Form 10-K, as required by Section 404 of the Sarbanes-Oxley Act. We will be required to perform this evaluation and testing of our internal control over financial reporting to allow management to report on the effectiveness of our internal control over financial reporting on an annual basis. This requires that we incur substantial additional professional fees and internal costs and that we expend significant management efforts on an annual basis. We have and will be required to test our internal control within a specified period, and, as a result, we may experience difficulty in meeting these reporting requirements.

We may discover weaknesses in our system of internal financial and accounting controls and procedures that could result in a material misstatement of our financial statements. Our internal control over financial reporting will not prevent or detect all errors and all fraud. A control system, no matter how well designed and operated, can provide only reasonable, not absolute, assurance that the control system's objectives will be met. Because of the inherent limitations in all control systems, no evaluation of controls can provide absolute assurance that misstatements due to error or fraud will not occur or that all control issues and instances of fraud will be detected.

For example, based on management's assessment using the framework set forth in the report entitled "*Internal Control – Integrated Framework (2013)*", in connection with the preparation of this Annual Report on Form 10-K, management identified a material weakness in internal controls related to having sufficient, documented policies and procedures in place to address significant and/or at-risk areas. As a result, in prior years, the Company did not appropriately account for warrants to purchase the Company's common stock. These warrants had not been appropriately accounted for as liabilities in prior periods, which led to understatements of derivative and warrant liability and additional paid-in capital and fluctuations in fair value adjustment related to derivative and warrant liability during the impacted periods.

The material weakness described above resulted in the Company restating its consolidated financial statements for the year ended December 31, 2022, and the Prior Interim Financial Statements in this Annual Report on Form 10-K. The material weakness did not result in any identified material misstatements in the consolidated financial statements as of and for the year ended December 31, 2023. As a result of the material weakness noted above, management has concluded that our internal control over financial reporting was not effective as of December 31, 2023.

If we are not able to comply with the requirements of Section 404 of the Sarbanes-Oxley Act, or if we are unable to maintain proper and effective internal control, we may not be able to produce timely and accurate financial statements. If that were to happen, the market price of our stock could decline and we could be subject to sanctions or investigations by the stock exchange on which our common stock is listed, the SEC or other regulatory authorities.

General Risk Factors

An active trading market for our common stock may not be sustained and you may not be able to resell your shares of our common stock for a profit, if at all.

An active trading market for our shares may not be sustained. If an active market for our common stock is not sustained, it may be difficult for you to sell our shares at an attractive price, or at all.

If equity research analysts do not publish research or reports, or publish unfavorable research or reports, about us, our business or our market, our stock price and trading volume could decline.

The trading market for our common stock is influenced by the research and reports that securities or industry analysts publish about us or our business, our market and our competitors. We do not have any control over these analysts. If one or more of the analysts who cover us downgrade our shares or change their opinion of our shares, our share price would likely decline. If one or more of these analysts cease coverage of our company or fail to regularly publish reports on us, we could lose visibility in the financial markets, which could cause our share price or trading volume to decline.

Because we do not anticipate paying any cash dividends on our common stock in the foreseeable future, capital appreciation, if any, will be your sole source of gains and you may never receive a return on your investment.

You should not rely on an investment in our common stock to provide dividend income. We have not declared or paid cash dividends on our common stock to date. We currently intend to retain our future earnings, if any, to fund the development and growth of our business. In addition, the terms of any future debt agreements may preclude us from paying dividends. As a result, capital appreciation, if any, of our common stock will be your sole source of gain for the foreseeable future. Investors seeking cash dividends should not purchase our common stock.

We incur increased costs and demands upon management as a result of being a public company.

As a public company listed in the United States, we incur significant additional legal, accounting and other costs. These additional costs could negatively affect our financial results. In addition, changing laws, regulations and standards relating to corporate governance and public disclosure, including regulations implemented by the SEC, may increase legal and financial compliance costs and make some activities more time consuming. These laws, regulations and standards are subject to varying interpretations and, as a result, their application in practice may evolve over time as new guidance is provided by regulatory and governing bodies. We intend to invest resources to comply with evolving laws, regulations and standards, and this investment may result in increased general and administrative expenses and a diversion of management's time and attention from revenue-generating activities to compliance activities. If, notwithstanding our efforts to comply with new laws, regulations and standards, we fail to comply, regulatory authorities may initiate legal proceedings against us.

Failure to comply with these rules might also make it more difficult for us to obtain some types of insurance, including director and officer liability insurance, and we might be forced to accept reduced policy limits and coverage or incur substantially higher costs to obtain the same or similar coverage. The impact of these events could also make it more difficult for us to attract and retain qualified persons to serve on our board of directors, on committees of our board of directors or as members of senior management.

Global economic uncertainty and other global economic or political and regulatory developments could have a material adverse effect on our business, cash flows, financial condition and/or prospects. Growth in the global pharmaceutical market has become increasingly tied to (i) global economic growth as an economic downturn may reduce the amount of funding for the pharmaceutical sector as a whole or certain diseases targeted by us and (ii) political conditions, tension and uncertainty which could, for instance, impact the regulations applicable to us. Uncertain political and geopolitical conditions currently exist in various parts of the world, such as the ongoing conflict between Russia and Ukraine. As a result, the United States and other governments have announced certain sanctions against Russia. The conflict in Ukraine and any retaliatory measures taken by the United States and other governments could threaten global security and result in further regional conflict and otherwise have a lasting impact on regional and global economies, any or all of which could adversely affect our business. In addition, the full effects of the United Kingdom's exit from the European Union in January 2020 are impossible to predict but may result in significant market volatility and dislocation, and adversely affect the United Kingdom, European and global economy.

Future legal or regulatory changes in jurisdictions where we currently operate, or in such jurisdictions in which we may choose to operate in the future, could materially and adversely affect our business, results of operations, cash flows, financial condition and/or prospects, including by imposing regulatory and operational restrictions and compliance obligations on our business, reducing our revenue or increasing our expenses.

The above circumstances, individually or in the aggregate, could have a material adverse effect on our business, cash flows, financial condition and/or prospects.

The increasing focus on environmental sustainability and social initiatives could increase our costs, harm our reputation and adversely impact our financial results.

There has been increasing public focus by investors, patients, environmental activists, the media and governmental and nongovernmental organizations on a variety of environmental, social and other sustainability matters. We may experience pressure to make commitments relating to sustainability matters that affect us, including the design and implementation of specific risk mitigation strategic initiatives relating to sustainability. If we are not effective in addressing environmental, social and other sustainability matters affecting our business, or setting and meeting relevant sustainability goals, our reputation and financial results may suffer. In addition, even if we are effective at addressing such concerns, we may experience increased costs as a result of executing upon our sustainability goals that may not be offset by any benefit to our reputation, which could have an adverse impact on our business and financial condition.

In addition, this emphasis on environmental, social and other sustainability matters has resulted and may result in the adoption of new laws and regulations, including new reporting requirements. If we fail to comply with new laws, regulations or reporting requirements, our reputation and business could be adversely impacted.

Adverse developments affecting the financial services industry, including events or concerns involving liquidity, defaults, or non-performance by financial institutions, could adversely affect our business, financial condition or results of operations.

Financial instruments that potentially subject the Company to a concentration of credit risk consist primarily of cash, cash equivalents, and investments. The Company invests in money market funds, U.S. treasury securities, and U.S. government agency securities. The Company maintains bank deposits in federally insured financial institutions and these deposits may exceed federally insured limits. The Company is exposed to credit risk in the event of a default by the financial institutions holding its cash and cash equivalents to the extent recorded on the consolidated balance sheets. Should events, including limited liquidity, defaults, non-performance or other adverse developments occur with respect to the banks or other financial institutions that hold our funds, or that affect financial institutions or the financial services industry generally, or concerns or rumors about any events of these kinds or other similar risks, our liquidity may be adversely affected. For example, on March 10, 2023, the Federal Deposit Insurance Corporation (FDIC) announced that Silicon Valley Bank had been closed by the California Department of Financial Protection and Innovation. Although we did not have any funds in Silicon Valley Bank or other institutions that have been closed, we cannot guarantee that the banks or other financial institutions that hold our funds will not experience similar issues.

ITEM 1B. UNRESOLVED STAFF COMMENTS

Not applicable.

ITEM 1C. CYBERSECURITY

Cybersecurity Risk Management and Strategy

We have developed and implemented a cybersecurity risk management program intended to protect the confidentiality, integrity, and availability of our critical systems and information. Our cybersecurity risk management program includes a cybersecurity incident response plan.

We design and assess our program based on the National Institute of Standards and Technology Cybersecurity Framework ("NIST CSF"). This does not imply that we meet any particular technical standards, specifications, or requirements, only that we use the NIST CSF as a guide to help us identify, assess, and manage cybersecurity risks relevant to our business.

Our cybersecurity risk management program is integrated into our overall enterprise risk management program, and shares common methodologies, reporting channels and governance processes that apply across the enterprise risk management program to other legal, compliance, strategic, operational, and financial risk areas.

Our cybersecurity risk management program includes:

- risk assessments designed to help identify material cybersecurity risks to our critical systems, information, products, services, and our broader enterprise IT environment;
- a security team principally responsible for managing (1) our cybersecurity risk assessment processes, (2) our security controls, and (3) our response to cybersecurity incidents;
- the use of external service providers, where appropriate, to assess, test or otherwise assist with aspects of our security controls;
- cybersecurity awareness training of our employees, incident response personnel, and senior management;
- a cybersecurity incident response plan that includes procedures for responding to cybersecurity incidents; and
- a third-party risk management process for service providers, suppliers, and vendors.

We have not identified risks from known cybersecurity threats, including as a result of any prior cybersecurity incidents, that have materially affected or are reasonably likely to materially affect us, including our operations, business strategy, results of operations, or financial condition.

Cybersecurity Governance

Our Board considers cybersecurity risk as part of its risk oversight function and has delegated to the Audit Committee ("Committee") oversight of cybersecurity and other information technology risks. The Committee oversees management's implementation of our cybersecurity risk management program.

The Committee receives periodic reports from management on our cybersecurity risks. In addition, management updates the Committee, as necessary, regarding any material cybersecurity incidents, as well as any incidents with lesser impact potential.

The Committee reports to the full Board regarding its activities, including those related to cybersecurity. The full Board also receives briefings from management on our cyber risk management program. Board members receive presentations on cybersecurity topics from our internal security staff or external experts as part of the Board's continuing education on topics that impact public companies. Our management team, including R. LaDuane Clifton, Chief Financial Officer, Secretary and Treasurer, is responsible for assessing and managing our material risks from cybersecurity threats. The team has primary responsibility for our overall cybersecurity risk management program and supervises both our internal cybersecurity personnel and our retained external cybersecurity consultants. Our management team includes IT staff with more than 40 years of combined experience in various roles involving managing information security, managing privacy and data protection, evaluating cybersecurity risks, developing cybersecurity strategy, and implementing cybersecurity programs.

Our management team supervises efforts to prevent, detect, mitigate, and remediate cybersecurity risks and incidents through various means, which may include briefings from internal security personnel; threat intelligence and other information obtained from governmental, public or private sources, including external consultants engaged by us; and alerts and reports produced by security tools deployed in the information technology environment.

ITEM 2. PROPERTIES

As of December 31, 2023, we have leased approximately 17,000 square feet of headquarters office space in Celebration, Florida, comprised of two contiguous office suites, under a non-cancelable lease agreement that expires in August 2025 and February 2026, respectively. One such office suite, which is approximately 6,300 square feet and has an expiration date of February 2026, is subleased to a third party. We have the right to extend the term of the lease for two successive five-year terms upon expiration. In addition, we occupy leased office space in Newton, Massachusetts and Copenhagen, Denmark, as well as leased laboratory space in Coralville, Iowa and Blacksburg, Virginia.We believe that our facilities are adequate for our current needs.

ITEM 3. LEGAL PROCEEDINGS

From time to time, we may be involved in routine legal proceedings, as well as demands, claims and threatened litigation, which arise in the normal course of our business. Following the Merger, we are now a party to certain legal proceedings to which Acer had previously been named as a party. Other than as disclosed in Note H of our audited consolidated financial statements included elsewhere in this Annual Report on Form 10-K, we believe there is no litigation pending that would reasonably be expected to, individually or in the aggregate, have a material adverse effect on our results of operations or financial condition.

ITEM 4. MINE SAFETY DISCLOSURES

Not applicable.

INFORMATION ABOUT OUR EXECUTIVE OFFICERS AND DIRECTORS

The following table sets forth information regarding our executive officers and directors as of the date of this Annual Report on Form 10-K.

Name	Age	Position
Executive Officers		
Neil F. McFarlane, M.S.	51	President, Chief Executive Officer, and Director
R. LaDuane Clifton, MBA, CPA	51	Chief Financial Officer, Secretary and Treasurer
Sven Guenther, Ph.D.	52	Chief Scientific Officer
Christal M.M. Mickle, M.A.	45	Chief Development Officer; Co-Founder
Joshua Schafer, MBA	53	Chief Commercial Officer and Executive Vice President of Business Development
Adrian Quartel, M.D., FFPM	63	Chief Medical Officer
Non-Employee Directors		
Thomas D. Anderson	68	Director
John B. Bode	49	Director
Douglas W. Calder	57	Director
Wendy Dixon, Ph.D.	68	Director
Tamara A. Favorito	65	Director
Alvin Shih, M.D.	47	Director
Corey Watton	54	Director

Executive Officers

Neil F. McFarlane

Neil F. McFarlane has served as our president, chief executive officer, and a director of our Company since October 2023. Mr. McFarlane has served as a member of the board of directors of Collegium Pharmaceutical Inc. since April 2022. Previously, Mr. McFarlane served as the chief executive officer and a member of the board of directors of Adamas Pharmaceuticals, Inc. from September 2019 until its acquisition by Supernus Pharmaceuticals, Inc. in November 2021. Mr. McFarlane worked as an independent consultant between June 2019 and August 2019 and again between December 2021 and September 2023 providing consulting services for biotechnology, pharmaceutical, and financial services companies. From August 2016 to May 2019, Mr. McFarlane served as the chief operating officer of Retrophin, Inc., now known as Travere Therapeutics, Inc., where he was responsible for overseeing operations. From 2011 to 2016 Mr. McFarlane served as vice president and general manager of UCB, Inc.'s U.S. Immunology Business Unit and as vice president for its Global Bone Business Unit in collaboration with Amgen Inc. Prior to his positions at UCB, Mr. McFarlane held positions of increasing responsibility with Genzyme Corporation ("Genzyme") and Sangstat Medical Corporation prior to its acquisition by Genzyme. Mr. McFarlane previously served as an officer and enlisted soldier in the United States Army Reserves. He received his B.S. degree and M.S. degree in Nursing from the University of Florida. Our Board believes that Mr. McFarlane's over 20 years of global biopharmaceutical and life sciences experience provide him with the qualifications and skills to serve as a director.

Christal M.M. Mickle, M.A.

Christal M.M. Mickle, M.A. has served as our chief development officer since January 2023. Ms. Mickle, who co-founded and has held a variety of positions at Zevra, also served as interim president and chief executive officer from June 2023 to October 2023. She previously served as senior vice president, operations and product development from June 2022 to January 2023. In this role, she managed the development of each of Zevra's products through strategic collaborations across the various drug development disciplines including clinical, regulatory, nonclinical, and manufacturing, enabling efficient use of funds and the ability to meet timelines and milestones. From January 2018 through June 2022, Ms. Mickle served as Zevra's vice president, product development and operations. Before co-founding Zevra in 2006, Ms. Mickle started her career as a research associate for New River Pharmaceuticals, preparing compounds in ADHD, pain, and thyroid dysfunctions for further study. Throughout her more than 20 years in the pharmaceutical industry, Ms. Mickle has been involved in early discovery as a medicinal chemist, starting and helping build a pharmaceutical company, and interacting with the FDA. In addition, her efforts managing a team of talented scientists has led to the approval of three NDAs. Ms. Mickle received her M.A. degree in Medicinal Chemistry from the University of Virginia and her B.A. and B.S. degrees in Chemistry and Biochemistry, respectively, from Virginia Polytechnic Institute and State University. She is also listed as an inventor on several patents.

Sven Guenther, Ph.D.

Sven Guenther, Ph.D. has served as our chief scientific officer since January 2023. Dr. Guenther was one of the first members of Zevra, he joined our Company as our group leader of researcl in 2007, and served as our executive vice president, research and development, from May 2014 to January 2023. In this role, he was a key contributor to the strategy and execution of all o Zevra's early discovery work, as well as, the development and approval of three NDAs. As chief scientific officer, he continues to lead Zevra's research team and play a central role in the advancement of the Company's pipeline. Dr. Guenther previously served as a research scientist for New River Pharmaceuticals, where he was part of the development team for VYVANSE. He earned his Ph.D. from the University of Iowa and is listed as an inventor on numerous patents, as well as an author of several research papers.

R. LaDuane Clifton, MBA, CPA

R. LaDuane Clifton, MBA, CPA, has served as our chief financial officer since June 2015 and as our secretary and treasurer since February 2016. He is responsible for the company's financial, operating and compliance activities. Previously, Mr. Clifton served as our vice president of finance and corporate controller from April 2015 to June 2015. Prior to joining our company, Mr. Clifton served in a variety of positions with The LGL Group, Inc., a publicly-traded producer of industrial and commercial products and services, from August 2009 to February 2015, including chief financial officer, secretary and treasurer from December 2012 to February 2015, chief accounting officer and secretary from March 2010 to December 2012, and corporate controller from August 2009 to March 2010. From August 2008 to August 2009, Mr. Clifton served as the chief financial officer of a21, Inc., a publicly-traded holding company with businesses in stock photography and the online retail and manufacturer of framed art, and as its corporate controller from March 2007 to August 2008. Mr. Clifton served in a variety of finance and medical cost analysis roles with Aetna, Inc., a publicly-traded provider of healthcare benefits, from August 1991 to August 2004. Mr. Clifton was an auditor with KPMG, LLP from August 2004 to March 2007. Mr. Clifton received his B.B.A. and M.B.A. degrees from the University of North Florida and is a certified public accountant in the state of Florida.

Joshua Schafer, M.S., MBA

Joshua Schafer, M.S., MBA, has served as our chief commercial officer and executive vice president, business development since January 2023. Mr. Schafer brings to Zevra over 25 years of pharmaceutical commercial, new product development and merger and acquisition ("M&A") experience. Mr. Schafer previously served as senior vice president and general manager of the Autoimmune and Rare Disease business at Mallinckrodt Pharmaceuticals from December 2020 to November 2022. Prior to that at Mallinckrodt Pharmaceuticals, he served as chief strategy and business officer from September 2019 to December 2020 and senior vice president of business development and general manager from January 2018 to September 2019. Prior to Mallinckrodt, Mr. Schafer served as vice president and oncology therapeutic area head, global marketing and strategy at Astellas Pharmaceuticals, where he was responsible for building the company's global oncology franchise, and also held senior roles at Takeda Pharmaceuticals, Accenture (formerly Anderson Consulting), G. D. Searle & Co. (later acquired by Pfizer) and Cognia Corporation. During his professional career, he has successfully led over $16 billion in aggregate M&A transactions. Mr. Schafer currently serves as a board member of Pharnext SA and Shuttle Pharmaceuticals. He received his B.A. in Biology and German at the University of Notre Dame, and both an M.S. in Biotechnology and an M.B.A. from Northwestern University

Adrian Quartel, M.D., FFPM

Adrian Quartel, M.D., FFPM, has served as our chief medical officer since January 2023, and previously served as the chief medical officer of Acer Therapeutics from February 2022 until January 2023, where he played a key role in guiding clinical development, medical affairs and regulatory compliance. Prior to that, he was the chief medical officer at Adamas Pharmaceuticals, a company focused on drug development for neurological diseases, from September 2020 until February 2022. From June 2017 until September 2020, Dr. Quartel served as the group vice president of global medical affairs at BioMarin Pharmaceuticals Inc., where he spearheaded the launch of six treatments for rare diseases or genetic disorders, including KUVAN, VIMIZIM, and BRINEURA. Before his tenure at BioMarin, Dr. Quartel oversaw clinical development and held senior medical leadership positions at Astellas Pharma, Inc. from January 2004 until September 2006, at Chiltern, a specialist contract research organization, from September 2006 to July 2007, and ICON Clinical Research from August 2001 to January 2004. In addition, Dr. Quartel worked as a clinical research fellow at UCLA Cedar Sinai and as a resident in cardio-thoracic surgery at Erasmus University Medical Center. He holds an M.D. from Erasmus University Medical School, Rotterdam, and has a postgraduate specialization in pharmaceutical medicine from the Faculty of Pharmaceutical Medicine in London. Dr. Quartel is board certified by the General Medical Council (GMC) in pharmaceutical medicine in the United Kingdom.

Directors

Thomas D. Anderson

Thomas D. Anderson has served as a director of our Company since August 2023. Mr. Anderson is a 35-year veteran of the biopharma industry and has led and been a part of high-growth organizations for much of his career. Mr. Anderson has served as chief executive officer and director of SwanBio Therapeutics since September 2019 until semi-retirement in October 2023, and subsequently resigned his board directorship in March 2024. Prior to that, he served as the chief commercial strategy officer of Sage Therapeutics, Inc. from 2014 to 2018. Between 2004 and 2014, Mr. Anderson was a senior operating executive with Shire Pharmaceuticals Group in a number of operational and strategic roles in both rare diseases and specialty pharmaceuticals. Prior to Shire, he spent 17 years at Johnson & Johnson's pharmaceutical companies, McNeil and Janssen, in various business capacities. Mr. Anderson is also an investor partner at Robin Hood Ventures in Philadelphia, Pennsylvania. Mr. Anderson earned his MBA from the University of Notre Dame's Mendoza College of Business Administration. He received his BS in civil engineering from the P.C. Rossin College of Engineering at Lehigh University. Our Board believes that Mr. Anderson's significant leadership experience in the biotechnology industry and his experience in rare disease qualifies him to serve on the Board.

John B. Bode

John B. Bode has served as a director of our Company since April 2023, and as the chair of the nominating and corporate governance committee since May 2023. Since February 2015, Mr. Bode has been the owner and managing director of Aerie Investments, LLC, an investment company focused on assisting legacy media companies and digital media start-ups with business development, strategic initiatives, and raising capital. Mr. Bode currently serves as the chief financial officer and chief transformation officer of Postmedia Network Canada Corp., a publishing company whose shares are traded on the Toronto Stock Exchange. Since September 2022, he has served as the interim chief executive officer of Fision Holdings, Inc. and as a member of its board of directors since March 2018. Mr. Bode's past corporate experience includes many years serving as a key executive and/or financial officer for leading public companies Prior to the founding of Aerie Investments, LLC, Mr. Bode was the chief financial officer of the Tribune Publishing Company from October 2013 to January 2015. From January 2011 to September 2013, Mr. Bode served as the chief financial officer of Source Interlink Companies, one of the largest enthusiast media companies in the United States and a leading distributor of periodicals, after serving in other accounting and finance roles with Source Interlink since 2002. Mr. Bode currently serves as on the board of The McClatchy Company, a leading privately-held publisher of newspapers, as well as of SPAR Group, Inc., a leading global provider of merchandising, marketing and distribution services that is listed on the Nasdaq. He was previously employed as a certified public accountant for BDO Seidman. Mr. Bode received a BS in accounting from Notre Dame University.

Douglas W. Calder

Douglas W. Calder has served as a director of our Company since April 2023. Since 2015, Mr. Calder has served as president and a director of Vycellix, Inc and its subsidiaries and affiliates. He has also served as a member of the board of directors for NextGenNK since June 2019; member of the board of directors of BioFlorida since January 2019, and a member of the Society for Natural Immunity since July 2018. Mr. Calder has more than 30 years of life science executive experience, having served in various senior executive roles for Florida-based biotechnology companies and research institutes including Viragen, Accentia Biopharmaceuticals, Biovest International and the Vaccine & Gene Therapy Institute of Florida, as well as having formerly served as a registered financial portfolio manager with a focus on life science equities with the New York Stock Exchange member firms, Gruntal & Co. and Dean Witter Reynolds. Mr. Calder received a BA from Florida State University.

Wendy Dixon, Ph.D.

Wendy Dixon, Ph.D. has served as a director of our Company since April 2023. Dr. Dixon has more than 40 years of biopharmaceutical industry experience in drug development with leadership roles in regulatory affairs and commercial capabilities. She currently serves on the board of directors of Arivinas, Inc. since June 2020, Black Diamond Therapeutics, Inc. since April 2022, and Iovance Biotherapeutics, Inc. since June 2022. Previously, Dr. Dixon has served on the boards of directors of Alkermes plc from January 2011 to May 2022, bluebird bio, Inc. from May 2013 to June 2021, Incyte from May 2010 to May 2022, Sesen Bio, Inc. (formerly Eleven Biotherapeutics, Inc.) from October 2014 to February 2020, Voyager Therapeutics, Inc. from January 2017 to January 2021, and and was formerly on the boards of Ardea Biosciences from 2011 until 2012 when Ardea was acquired by AstraZeneca plc, Furiex Pharmaceuticals from 2010 until 2014 when Furiex was acquired by Actavis plc, Dentsply International from July 2005 to July 2010, and Orexigen Therapeutics, Inc. from April 2010 until January 2016. From December 2001 to May 2009, Dr. Dixon was chief marketing officer and president of global marketing at Bristol Myers Squibb, and served as a senior vice president of marketing at Merck from 1996 to 2001. Earlier in her career, she held executive management positions at West Pharmaceuticals, Osteotech and Centocor, as well as roles at SmithKline and French (now GlaxoSmithKline) in marketing, regulatory affairs, project management and as a biochemist. Dr. Dixon holds a Ph.D. in biochemistry and an M.Sc. and B.Sc. in Natural Science from the University of Cambridge.

Tamara A. Favorito

Tamara A. Favorito has served as a director and chair of the audit committee of our Company since August 2021, and as our board chair since May 2023. Ms. Favorito has more than 30 years of life sciences industry experience including 20 years as a chief financial officer. She currently serves as a board member and audit committee chair of Artelo Biosciences, Inc. since March 2021 and Kintara Therapeutics, Inc. since April 2021, both publicly-traded clinical-development stage companies. Ms. Favorito served on the board of directors of Beacon Discovery, Inc. from 2018 until their acquisition in March 2021. Ms. Favorito was interim chief financial officer of Immunic, Inc., a publicly-traded clinical-stage drug development company in 2019. She served as chief financial officer of Signal Genetics, Inc. (now Viridian Therapeutics, Inc.), a publicly-traded molecular diagnostics company, from 2014 to 2017, HemaQuest Pharmaceuticals, Inc., a venture backed clinical-stage drug development company, from 2010 to 2014 and Favrille, Inc. (now MMR Global, Inc.), a publicly-traded clinical-stage drug development company, from 2001 to 2009. Earlier in her career, she spent eight years in public accounting with Deloitte & Touche LLP and PricewaterhouseCoopers LLP. Ms. Favorito is a certified public accountant (inactive). She received an MBA, emphasis in Finance, from Georgia State University, and a BBA, emphasis in Accounting, from Valdosta State University. Our Board believes that Ms. Favorito's deep experience in corporate management, finance and life science as chief financial officer of multiple public companies provides her with the qualifications and skills to serve as a director of our Company. Her extensive experience includes leading multiple private and public financings and M&A transactions as well as leading the finance, investor relations, human resources administration and managed care functions.

Alvin Shih, M.D.

Alvin Shih, M.D. has served as a director of our Company since January 2024. Dr. Shih has broad experience in drug development, spanning multiple indications with a focus on rare diseases, including as the chief operating officer and founding member of Pfizer Inc.'s rare disease research unit from May 2010 to May 2014. Most recently, he has served as president and chief executive officer of Catamaran Bio, Inc. since February 2021. Prior to his current role, from July 2019 to December 2020 he served as the chief executive officer of Disarm Therapeutics, a biotechnology company that developed therapeutics for neurodegenerative diseases and that was acquired by Eli Lilly in October 2020. Before that, Dr. Shih was chief executive officer of Enzyvant Therapeutics from November 2016 to February 2019, where he led the company's cell/tissue-based therapy development for treating a rare immunological disease. He was also the executive vice president and head of research at Retrophin, Inc. where he worked on therapies for multiple disease indications. Dr. Shih previously worked in management consulting at McKinsey & Company and L.E.K. Consulting, LLC. He received his medical degree from the University of Alabama and completed his residency training at Massachusetts General Hospital. Dr. Shih received his M.B.A. from the Kellogg School of Management at Northwestern University. The Board believes that Dr. Shih’s significant leadership experience in the biotechnology industry and his experience in rare disease qualifies him to serve on the Board.

Corey Watton

Corey Watton has served as a director of our Company since April 2023. Mr. Watton currently serves as president and chief executive officer of Fusion Medical Staffing, LLC, since September 2023. Prior to his CEO role, Mr. Watton served as chief financial officer from August 2019 until September 2023. In that role he oversaw finance, project management, operations, facilities and legal, and previously oversaw human resources as well. From April 2009 through July 2019, he served as the chief financial officer of Home Instead Senior Health Care, a global leader in in-home health care, where he oversaw finance, subsidiary companies and the financial arm of an international subsidiary. He previously was a partner of Lutz & Company, a regional accounting firm from 1996 to 2009. Mr. Watton is a certified public accountant and received a BS in Finance and Accounting from University of Nebraska-Lincoln.

PART II

ITEM 5. MARKET FOR REGISTRANT'S COMMON EQUITY, RELATED STOCKHOLDER MATTERS, AND ISSUER PURCHASES OF EQUITY SECURITIES

Common Stock Listing

On March 1, 2023, our common stock began trading on The Nasdaq Global Select Market under the ticker symbol "ZVRA". Prior to that, on October 19, 2021, our common stock began trading on The Nasdaq Global Select Market under the ticker symbol "KMPH". Prior to that date, our common stock was listed on the Nasdaq Capital Market under the ticker symbol "KMPH".

Holders of our Common Stock

As of December 31, 2023, there were approximately 96 holders of record of our common stock. The actual number of stockholders is greater than this number of record holders, and includes stockholders who are beneficial owners, but whose shares are held in street name by brokers and other nominees. This number of holders of record also does not include stockholders whose shares may be held in trust by other entities.

Dividend Policy

We have never declared or paid any cash dividends on our common stock. We anticipate that we will retain all of our future earnings, if any, for use in the operation and expansion of our business and do not anticipate paying cash dividends in the foreseeable future.

Securities Authorized for Issuance under Equity Compensation Plans

The information regarding securities authorized for issuance under equity compensation plans is included in Part III of this report.

Recent Sales of Unregistered Securities

None.

Purchases of Equity Securities By the Issuer and Affiliated Purchasers

None.

ITEM 6. [Reserved]

ITEM 7. MANAGEMENT'S DISCUSSION AND ANALYSIS OF FINANCIAL CONDITION AND RESULTS OF OPERATIONS

You should read the following discussion and analysis of our financial condition and results of operations together with our financial statements and related notes thereto included elsewhere in this Annual Report on Form 10-K. Some of the information contained in this discussion and analysis or set forth elsewhere in this Annual Report on Form 10-K, including information with respect to our plans and strategy for our business and related financing, includes forward-looking statements that involve risks and uncertainties. As a result of many factors, including those factors set forth in the "Risk Factors" section of this Annual Report on Form 10-K, our actual results could differ materially from the results described in or implied by the forward-looking statements contained in the following discussion and analysis.

This Part II, Item 7 includes restated financial data. See "*Explanatory Note.*"

Overview

We are a rare disease company combining science, data and patient need to create transformational therapies for diseases with limited or no treatment options. Our mission is to bring life changing therapeutics to people living with rare diseases. With unique, data-driven development and commercialization strategies, we are overcoming complex drug development challenges to make new therapies available to the rare disease community. We have a diverse portfolio of products and product candidates, which includes preclinical developmental programs, clinical stage pipeline and commercial stage assets. Our team has specialized expertise and a track record of success in advancing promising therapies that face complex clinical and regulatory challenges with an approach that balances science and data with patient need.

Following the U.S. approval of AZSTARYS® (further described below) in March 2021, we undertook a strategic process to evaluate how to leverage and potentially augment the Company's existing capabilities while also considering where to invest in our pipeline to generate long-term shareholder value. With a track record of drug development success leading to approvals for products which had either difficult pathways to approval or where approvals were won following a complete response letter ("CRL") from the U.S. Food and Drug Administration ("FDA)", the Company determined to focus its expertise on rare disease indications, as well as seeking value-creating opportunities by building and directly commercializing product candidates in lieu of an out-licensing model. We are executing on this balanced approach by building a culture that is patient-focused and driven by our commitment to developing and making available therapies which address the myriad unmet needs within the rare disease community.

As part of our commitment to serving the rare disease community, in February 2023, we changed our name to Zevra Therapeutics, Inc. Our name, Zevra, is the Greek word for zebra, which is the internationally recognized symbol for rare disease. This name reflects our intense focus and dedication to developing transformational, patient-focused therapies for rare diseases with limited or no treatment options available, or treatment areas with significant unmet needs.

In May 2022, we purchased all of the assets and operations of Orphazyme A/S related to arimoclomol, settled all of Orphazyme's actual outstanding liabilities to its creditors with a cash payment of $12.8 million, and agreed to assume an estimated reserve liability of $5.2 million related to revenue generated from Orphazyme's Expanded Access Program in France (the "Arimoclomol EAP").

In order to accomplish our mission, we are seeking to further expand our pipeline through both internal development and through our business development activities to collaborate, partner, and potentially acquire additional assets. We intend to target assets that will allow us to leverage the expertise and infrastructure that we have built in order to mitigate risk and enhance our probability of success. In addition, we may consider external opportunities within neurology and neurodegenerative diseases, psychiatric disorders, and other rare diseases, along with adjacent or related therapeutic categories. If we are successful, expanding our pipeline could be accretive to our value proposition and has the potential to create incremental long-term value for stockholders.

On November 17, 2023, Zevra completed the acquisition of Acer. Pursuant to the Merger Agreement, Acer continues as a wholly-owned subsidiary of Zevra. The Merger included the acquisition of OLPRUVA® (sodium phenylbutyrate) for oral suspension, which was approved by the U.S. Food and Drug Administration (FDA) on December 27, 2022, for the treatment of urea cycle disorders ("UCDs"). Acer also had a pipeline of investigational product candidates, including celiprolol for the treatment of vascular Ehlers-Danlos syndrome ("vEDS") in, patients with a confirmed type III collagen (COL3A1) mutation. At the effective time of the Merger (the "Effective Time"), each share of common stock of Acer, par value $0.0001 per share, issued and outstanding immediately prior to the Effective Time (excluding cancelled shares and any shares held by holders who have exercised their appraisal rights) were converted into the right to receive (i) 0.1210 fully paid and non-assessable shares of common stock of Zevra, par value $0.0001 per share, and (ii) one non-transferable contingent value right ("CVR") issued by Zevra, which represents the right to receive one or more contingent payments up to an additional $76.0 million upon the achievement, if any, of certain commercial and regulatory milestones for Acer's OLPRUVA and celiprolol products within specified time periods. Certain additional cash payments are also possible pursuant to the CVRs with respect to milestones involving Acer's early-stage program ACER-2820 (emetine).

Our recurring operating losses and negative cash flows from operations raise substantial doubt about our ability to continue as a going concern. We have no current source of revenues to sustain our present activities. Accordingly, our ability to continue as a going concern will require us to obtain additional financing to fund our operations. The perception of our inability to continue as a going concern may make it more difficult for us to obtain financing for the continuation of our operations and could result in the loss of confidence by investors, suppliers and employees. Adequate additional financing may not be available to us on acceptable terms, or at all. To the extent that we raise additional capital through the sale of equity or debt, the terms of these securities may restrict our ability to operate. If we raise additional funds through collaborations, strategic alliances or marketing, distribution or licensing arrangements with third parties, we may be required to relinquish valuable rights. If we are unable to raise capital when needed or on attractive terms, we could be forced to delay, reduce or altogether cease our research and development programs or future commercialization efforts.

On September 16, 2020, the previous sponsor of the arimoclomol program, Orphazyme, submitted a new drug application, or NDA, seeking approval for arimoclomol to treat NPC. In June 2021, the FDA issued a complete response letter, or CRL, which means the FDA determined that it could not approve the NDA in its present form. Our aim is to prepare and resubmit an NDA that presents meaningful evidence of safety and efficacy of arimoclomol for its intended use. To that end, we are continuing to work diligently to characterize the substantial data generated since the CRL, including the recently completed four-year open-label safety trial which was recently presented at the 19th World*Symposium*™ in February 2023. Results from this analysis, based on up to four years of continuous treatment, suggest that arimoclomol may reduce the long-term progression of NPC. Upon fulfilling the randomized double-blinded portion of the phase 2/3 clinical trial, both placebo- and arimoclomol-treated patients were given the option to continue into the four-year (48 month) open-label-extension, or OLE, phase of the study with arimoclomol treatment provided in addition to their current standard of care. Progression of NPC disease through the DB and OLE phases was assessed utilizing the five-domain NPC Clinical Severity Scale (5DNPCCSS) and compared with an estimated progression calculated from the combination of untreated patients from the NPC-001 observational trial and placebo patients from the NPC-002 Phase 2/3 trial. We are also investigating correlations between relevant 5DNPCCSS domains and corresponding Scale for the Assessment and Rating of Ataxia, or SARA, test items to potentially provide further supportive evidence for 5DNPCCSS validity as a tool for evaluating NPC progression. The SARA test evaluates impairment related to cerebellar ataxia, which was a secondary endpoint in the Phase 2/3 clinical trial of arimoclomol in NPC (NPC progression based on the 5DNPCCSS was the primary endpoint). Based on a comparative analysis of both measurements, it was determined that individual 5DNPCCSS domains and relevant performance-based SARA test items showed strong associations and alignment between the two instruments for all analysis methods used. These results provide further support that the evaluated 5DNPCCSS domains are appropriately standardized to allow for reliable and reproducible scoring of disease severity in NPC. In preparation of the arimoclomol NDA, we completed a productive and collaborative meeting with the FDA in August 2023, receiving important information that was used to finalize the NDA submission. The arimoclomol NDA was submitted to the FDA on December 21, 2023, and is currently undergoing review by the FDA. The FDA has assigned a PDUFA action date of September 21, 2024. We believe that, if approved by the FDA, arimoclomol will be eligible to receive a Rare Pediatric Disease Priority Review Voucher.

We also intend to advance our pipeline of prodrug product candidates for the treatment of IH and other CNS/rare diseases, and we reported top-line data from a Phase 1 proof-of-concept study of SDX in the fourth quarter of 2021 and final data for the Phase 1 proof-of-concept study of SDX in the first quarter of 2022. The proof-of-concept study was a dose-escalation study to evaluate the pharmacokinetics, pharmacodynamic stimulant effects, and safety of single oral doses of SDX in subjects with a history of high-dose stimulant use. In the trial, 240 mg and 360 mg doses of SDX were observed to be well-tolerated and produced d-MPH exposure that appeared to increase proportionally with dose. Mean d-MPH plasma concentrations showed a gradual increase after SDX administration, reaching a broad peak from eight to twelve hours post-dose, followed by a shallow decline thereafter. Increased wakefulness, alertness, hypervigilance, and insomnia effects were reported by study participants, which we believe suggests that SDX produced targeted pharmacodynamic effects that have the potential to benefit patients with IH and other sleep disorders. On November 18, 2022, we announced that the FDA has granted the orphan drug designation to SDX for the treatment of IH.

In January 2022, we announced that we had selected KP1077 for the treatment of IH and narcolepsy as our lead clinical development candidate. KP1077 utilizes SDX, our prodrug of d-MPH, as its API. During the first quarter of 2022, we initiated a Phase 1 clinical trial comparing the cardiovascular safety of SDX to immediate-release and long acting formulations of RITALIN®, a commonly prescribed CNS stimulant. In September 2022, we announced topline data from our exploratory Phase 1 clinical trial, which showed the potential for higher dose formulations of SDX to be safe and well tolerated while avoiding the potential for greater cardiovascular safety risk compared to immediate-release and long-acting formulations of Ritalin.

Based on the data, in December 2022, we announced the initiation of a double-blind, placebo-controlled, randomized-withdrawal, dose-optimizing, multi-center Phase 2clinical trial evaluating the efficacy and safety of KP1077 for the treatment of IH. The trial concluded in March 2024 and provided meaningful information of the optimal dose and dosing regimen to inform Phase 3 trial design.

We enrolled 48 adult patients with IH in more than 30 centers in the United States. Part 1 of the trial consisted of a five-week open-label titration phase during which patients were optimized to one of four doses of SDX (80, 160, 240, or 320 mg/day). Part 2 of the trial entailed a two-week randomized, double-blind, withdrawal phase, during which two-thirds of the trial participants will continue to receive their optimized dose while the remaining one-third will receive placebo. Participants were further assigned into two evenly divided cohorts. The first cohort received a single daily dose just before bedtime, and the second cohort received half the daily dose shortly after awakening and half the daily dose prior to bedtime.

Clinically meaningful improvements were observed across all studied endpoints. The exploratory endpoints of sleep inertia and brain fog performed in-line with expectations and were stable when compared across a variety of other endpoints. Symptom improvements in patients receiving KP1077 were similar after both once-per-day, and twice per-day dosing.

In the Phase 2 trial, KP1077 was observed to be well-tolerated at all dose levels and both dosing regimens, with adverse events that are typical for stimulants and mostly mild in severity. These results are consistent with data from the Phase 1 trial with serdexmethylphenidate (SDX) that indicated no greater cardiovascular safety risk despite higher overall exposure levels when compared to both immediate and long-acting methylphenidate products currently used off-label for the treatment of IH.

In the second quarter of 2023, we initiated a Phase 1 clinical trial in healthy volunteers to assess proposed dosing regimen for the narcolepsy indication. This study was completed in September 2023. By leveraging the data from the IH program, Zevra is evaluating the potential to initiate a Phase 3 trial in narcolepsy.

On August 30, 2023, Zevra and Merger Sub entered into the Merger Agreement with Acer. On the Closing Date, Zevra completed the acquisition of Acer. Pursuant to the Merger Agreement, on the Closing Date, Merger Sub was merged with and into Acer, with Acer continuing as the surviving entity and as a wholly-owned subsidiary of Zevra. The Merger included the acquisition of OLPRUVA® (sodium phenylbutyrate) for oral suspension, which was approved by the FDA on December 27, 2022, for the treatment of urea cycle disorders, or UCDs. Acer also has a pipeline of investigational product candidates, including celiprolol for the treatment of vascular Ehlers-Danlos syndrome, or vEDS, patients with a confirmed type III collagen (COL3A1) mutation.

At the Effective Time, each share of common stock of Acer, par value $0.0001 per share, issued and outstanding immediately prior to the Effective Time (excluding cancelled shares and any shares held by holders who have exercised their appraisal rights) were converted into the right to receive (i) 0.1210 fully paid and non-assessable shares of common stock of Zevra, par value $0.0001 per share, and (ii) one non-transferable CVR issued by Zevra, which represents the right to receive one or more contingent payments up to an additional $76.0 million upon the achievement, if any, of certain commercial and regulatory milestones for Acer's OLPRUVA and celiprolol products within specified time periods. Certain additional cash payments are also possible pursuant to the CVRs with respect to milestones involving Acer's early-stage program ACER-2820 (emetine).

As discussed further in "Liquidity and Capital Resources," in connection with the Merger, Zevra also purchased Acer's secured debt from Nantahala Capital Management, LLC ("NCM"), certain of its affiliates and certain other parties (collectively with NCM, "Nantahala") through a series of transactions and Zevra agreed to provide Acer with a bridge loan facility for up to $18.0 million ("Bridge Loan"), subject to certain terms and conditions.

To commercialize OLPRUVA for oral suspension in the U.S. we are building marketing, sales, medical affairs, distribution, managerial and other non-technical capabilities or making arrangements with third parties to perform these services. During the quarter ended December 31, 2023, we began generating revenue from the sale of OLPRUVA in the U.S. For additional information regarding the Merger, see Note R of our audited consolidated financial statements included elsewhere in this Annual Report on Form 10-K.

We have historically had minimal positive net cash flows from operations. Our cash flows used in operations for the years ended December 31, 2023, and 2022, were $33.0 million and $18.7 million, respectively.
As described elsewhere in this Annual Report on Form 10-K, our recurring operating losses and negative cash flows from operations raise substantial doubt about our ability to continue as a going concern.

We expect to continue to incur significant expenses and minimal positive net cash flows from operations or negative net cash flows from operations for the near future, and those expenses and losses may fluctuate significantly from quarter-to-quarter and year-to-year. We anticipate that our expenses will fluctuate substantially as we:

- continue to integrate the operations of Acer following the recent Merger;
- continue building and maintaining our ongoing commercial capabilities to support the launch of our approved product OLPRUVA® and, if approved, the commercial launch of arimoclomol in the U.S.
- continue our ongoing preclinical studies, clinical trials and our product development activities for our pipeline of product candidates;
- seek regulatory approvals for any product candidates that successfully complete clinical trials;
- continue research and preclinical development and initiate clinical trials of our product candidates;
- seek to discover and develop additional product candidates either internally or in partnership with other pharmaceutical companies;
- adapt our regulatory compliance efforts to incorporate requirements applicable to marketed products;
- maintain, expand and protect our intellectual property portfolio;
- incur additional legal, accounting and other expenses in operating as a public company; and
- add operational systems and personnel, if needed, to support any future commercialization efforts.

Third-Party Agreements

AZSTARYS License Agreement

See Part I, Item 1. "Business—AZSTARYS" of this Annual Report on Form 10-K for information regarding the AZSTARYS License Agreement.

Relief License Agreement

On August 28, 2023, as a condition to the Merger Agreement, Acer and Relief Therapeutics Holding AG ("Relief") entered into an agreement ("the Relief License Agreement"), pursuant to which Acer and Relief mutually agreed to terminate a pre-existing collaboration agreement. See "Liquidity and Capital Resources."

Other Third-Party Agreements

Under our March 2012 termination agreement with Aquestive, Aquestive has the right to receive a royalty amount equal to 10% of any value generated by AZSTARYS and any product candidates containing SDX. In connection with the AZSTARYS License Agreement, we paid Aquestive a royalty equal to 10% of the regulatory milestone and royalty payments we received in 2021 from AZSTARYS.

In July 2020, we entered into the Corium Consulting Agreementunder which Corium and Commave, respectively, engaged us to guide the product development and regulatory activities for certain current and potential future products in their portfolio, as well as continue supporting preparation for the potential commercial launch of AZSTARYS. Under the Corium Consulting Agreement, we received payments from Corium of up to $15.6 million, $13.6 million of which was paid in quarterly installments through March 31, 2022. The remaining $2.0 million was received in the first quarter of 2022 upon approval by the FDA of the NDA for Corium's product known as ADLARITY®.

Our current single distributor for sales of our approved product, OLPRUVA is a specialty pharmacy provider, however the Company intends to establish additional distributors such as other retail pharmacies and certain medical centers or hospitals. In addition to distribution agreements, we enter into arrangements with health care providers and payors that provide for government mandated and/or privately negotiated rebates with respect to the purchase of our products.

Components of our Results of Operations

Revenue

Our commercial revenue is, and will be, primarily derived from sales of our approved products or any of our product candidates for which we obtain regulatory approval, and sales of arimoclomol under the Arimoclomol EAP. We expect that our other sources of revenues will be through payments arising from our license agreements with Corium, and through any other future arrangements related to one of our product candidates. To date, we have generated revenue from the AZSTARYS License Agreement, sale of arimoclomol under the Arimoclomol EAP, reimbursement of out-of-pocket third-party costs, and the performance of consulting services. We cannot guarantee that either Corium will be able to successfully commercialize AZSTARYS or our product candidates covered under the AZSTARYS License Agreement, and we cannot guarantee that we will be able to successfully commercialize OLPRUVA. We also do not know when, if ever, any other product candidate will be commercially available.

Cost of Revenue

The components of our cost of revenue are royalties and expenses directly attributable to revenue. To date, we have generated revenue from the AZSTARYS License Agreement, sales of arimoclomol under the Arimoclomol EAP, reimbursement of out-of-pocket third-party costs and the performance of consulting services. In connection with the AZSTARYS License Agreement, we paid Aquestive a royalty equal to 10% of the upfront license payment and all regulatory milestone and royalty payments. In addition, we capitalized incremental costs directly attributable to the AZSTARYS License Agreement, these costs are amortized to royalties and contract costs as revenue is recognized.

Operating Expenses

We classify our operating expenses into two categories: research and development expenses and selling general and administrative expenses. Salaries and personnel-related costs, including benefits, bonuses and stock-based compensation expense, comprise a significant component of each of these expense categories. We allocate expenses associated with our facilities, information technology costs and depreciation and amortization between research and development expenses and general and administrative expenses based on employee headcount and the nature of work performed by each employee.

Research and Development Expense

Research and development expense consists of expenses incurred while performing research and development activities to discover and develop potential product candidates. This includes conducting preclinical studies and clinical trials, manufacturing development efforts and activities related to regulatory filings for product candidates. We recognize research and development expenses as they are incurred. Our research and development expense primarily consists of:

- salaries and personnel-related costs, including benefits and any stock-based compensation, for our scientific personnel performing research and development activities;
- costs related to executing preclinical studies and clinical trials;
- fees paid to consultants and other third parties who support our product candidate development;
- other costs in seeking regulatory approval of our products; and
- allocated facility-related costs and overhead.

We typically use our employee, consultant and infrastructure resources across our development programs. We track outsourced development costs by product candidate or development program, but we do not allocate personnel costs, other internal costs or external consultant costs to specific product candidates or development programs.

The following table summarizes our research and development costs for the years ended December 31, 2023, and 2022 (in thousands):

	Year Ended December 31,	
	2023	2022
		(As Restated)
Outsourced development costs directly identified to programs:		
Arimoclomol	$ 5,579	$ 2,940
KP1077	14,458	5,325
APADAZ (1)	2,446	145
Celiprolol	159	—
Total outsourced development costs directly identified to programs	22,642	8,410
Research and development costs not directly identified to programs:		
Personnel costs including cash compensation, benefits and stock-based compensation	11,970	7,709
Facilities costs	775	575
Other costs	4,419	3,109
Total research and development costs not directly allocated to programs	17,164	11,393
Total research and development expenses	$ 39,806	$ 19,803

(1) On May 31, 2023, Zevra and KVK-Tech, Inc. ("KVK") terminated the Collaboration and License Agreement that the parties entered into on October 25, 2018. In conjunction with the termination of the Collaboration and License Agreement, we agreed to pay a settlement to KVK of $0.9 million, which is included in research and development in the consolidated statement of operations for the year ended December 31, 2023, and was paid in October 2023.

We anticipate that our research and development expenses will fluctuate for the foreseeable future as we continue our efforts to advance the development of our product candidates, subject to the availability of additional funding. In accordance with the AZSTARYS License Agreement, Corium also agreed to be responsible and reimburse us for all of development, commercialization and regulatory expenses for the Licensed Product Candidates, subject to certain limitations as set forth in the AZSTARYS License Agreement.

The successful commercialization of AZSTARYS, OLPRUVA and any of our product candidates that may be approved and the development of our product candidates is highly uncertain. At this time, we cannot reasonably estimate the nature, timing or costs required to commercialize AZSTARYS, OLPRUVA or any of our product candidates, if approved, and complete the remaining development of any product candidates. This is due to the numerous risks and uncertainties associated with the commercialization and development of our products and product candidates.

Selling, General and Administrative Expense

General and administrative expenses primarily consist of salaries and personnel-related costs, including employee benefits and any stock-based compensation, for employees performing functions other than research and development. This includes personnel in executive, finance, human resources and administrative support functions. Other general and administrative expenses include facility-related costs not otherwise allocated to research and development expense, professional fees for auditing, tax and legal services, expenses associated with obtaining and maintaining patents, consulting costs and costs of our information systems.

We expect that our general and administrative expenses will fluctuate as we continue to operate as a public reporting company and continue to develop our product candidates. We believe that these fluctuations will likely include costs related to the hiring of additional personnel and fees for outside consultants, lawyers and accountants. We also expect to continue to incur costs to comply with corporate governance, internal control, investor relations, disclosure and similar requirements applicable to public reporting companies.

Other (Expense) Income

Other (expense) income consists primarily of non-cash costs associated with fair value adjustments to our derivative and warrant liability and amortization of debt issuance costs and debt discount to interest expense. Other (expense) income also includes interest expense incurred on our outstanding borrowings as well as interest and other income consisting primarily of interest earned on investments. These items are unrelated to our core business and thus are recognized as other (expense) income in our consolidated statements of operations.

Income Tax (Expense) Benefit

Income tax (expense) benefit consists of refundable state income tax credits and adjustments to those credits. To date, we have not been required to pay U.S. federal or state income taxes or Denmark taxes because we have not generated taxable income. We have received state income tax credits related to our qualified research activities in Iowa and Denmark. These refundable state income tax credits and adjustments to those credits are recognized as income tax expense (benefit) in our consolidated statements of operations, and to the extent the refundable state income tax credits are not collected as of period end, they are recognized as accounts receivable in our consolidated balance sheets.

Results of Operations

The results of operations and changes in stockholders' equity for Acer were included in the Company's consolidated financial statements beginning November 18, 2023. Acer had total operating revenue of $42,000 and a net loss of $6.8 million for the period from November 18, 2023, through December 31, 2023.

Comparison of the Years Ended December 31, 2023, and 2022 (in thousands):

	Year Ended December 31,		Period-to Period Change
	2023	2022	
		(As Restated)	
Revenue, net	$ 27,461	$ 10,161	$ 17,300
Operating expenses:			
Cost of revenue	2,945	222	2,723
Research and development	39,806	19,803	20,003
Selling, general and administrative	34,314	15,038	19,276
Acquired in-process research and development	—	17,663	(17,663)
Total operating expenses	77,065	52,726	24,339
Loss from operations	(49,604)	(42,565)	(7,039)
Other (expense) income:			
Interest expense	(1,501)	(335)	(1,166)
Fair value adjustment related to derivative and warrant liability and CVR liability	(98)	15,159	(15,257)
Fair value adjustment related to investments	613	(577)	1,190
Interest and other income, net	4,541	1,513	3,028
Total other income	3,555	15,760	(12,205)
Loss before income taxes	(46,049)	(26,805)	(19,244)
Income tax (expense) benefit	—	33	(33)
Net loss	$ (46,049)	$ (26,772)	$ (19,277)

Net Loss

Net loss for the year ended December 31, 2023, was $46.0 million compared to net loss of $26.8 million for the year ended December 31, 2022. The change was attributable to a decrease in the change in fair value adjustment related to derivative and warrant liability and CVR liability of $15.3 million, loss from operations of $7.0 million, partially offset by an increase in net interest income and other income of $3.0 million. There was no acquired in-process research and development expense in the year ended December 31, 2023, compared to $17.7 million for the year ended December 31, 2022, which in that year resulted from the acquisition of an intangible asset related to arimoclomol. The portion of the purchase that was allocated to in-process research and development assets acquired from Orphazyme was immediately expensed in accordance with ASC Subtopic 730-10-25, *Accounting for Research and Development Costs.*

Revenue

Revenue for the year ended December 31, 2023, was $27.5 million, an increase of $17.3 million compared to revenue of $10.2 million for the year ended December 31, 2022. The increase was primarily attributable to an increase in revenue from the AZSTARYS License Agreement of approximately $18.5 million, an increase in sales under the Arimoclomol EAP of approximately $3.3 million, partially offset by a decrease in revenue from the Corium Consulting Agreement of approximately $4.5 million.

Cost of Revenue

Cost of revenue for the year ended December 31, 2023, was $2.9 million, an increase of $2.7 million compared to cost of revenue of $0.2 million for the year ended December 31, 2022. The increase was primarily attributable to an increase in royalty payments related to revenue from the AZSTARYS License Agreement of approximately $1.9 million and an increase in amortization expense related to OLPRUVA of $0.8 million.

Research and Development

Research and development expenses increased by $20.0 million, from $19.8 million for the year ended December 31, 2022, to $39.8 million for the year ended December 31, 2023. This increase was attributable to an increase in third-party research and development costs of $14.5 million, an increase in other research and development costs of $2.9 million and an increase in personnel-related costs of $2.6 million.

General and Administrative

General and administrative expenses increased by $19.3 million, from $15.0 million for the year ended December 31, 2022, to $34.3 million for the year ended December 31, 2023. This increase was attributable to an increase in professional fees of $8.7 million and an increase in personnel-related costs of $9.7 million and an increase in other expenses of $0.9 million. Acer transaction costs were $2.2 million and are included in general and administrative expenses.

Acquired in-process research and development

There was no acquired in-process research and development expense in the year ended December 31, 2023, compared to $17.7 million for the year ended December 31, 2022, which in that year resulted from the acquisition of an intangible asset related to arimoclomol.

Other Income

Other income decreased by $12.2 million, from $15.8 million expense for the year ended December 31, 2022, to $3.6 million of income for the year ended December 31, 2023. This period-to-period decrease in income was primarily attributable to a decrease in the fair value adjustment related to derivative and warrant liability and CVR liability of $15.3 million and an increase in interest expense of $1.1 million, partially offset by an increase in net interest and other income of $3.0 million and an increase in the change in fair value related to investments of $1.2 million.

Liquidity and Capital Resources

Sources of Liquidity

Through December 31, 2023, we have funded our research and development and operating activities primarily through the issuance of debt, private placements of redeemable convertible preferred stock and the sale of common stock in our initial public offering, at-the-market offering, underwritten public offerings, through our purchase agreements with Lincoln Park Capital LLC, or Lincoln Park, and from revenue received under the Arimoclomol EAP, AZSTARYS License Agreement, the Corium Consulting Agreement and other consulting arrangements. As of December 31, 2023, we had cash, cash equivalents and investments of $67.7 million.

To date, we have generated revenue from the Arimoclomol EAP, AZSTARYS License Agreement, reimbursement of out-of-pocket third-party costs, the performance of consulting services, and sales of OLPRUVA.

In July 2020, we entered into the Corium Consulting Agreement under which Corium and Commave, respectively, engaged us to guide the product development and regulatory activities for certain current and potential future products in their portfolio, as well as continue supporting preparation for the potential commercial launch of AZSTARYS. Under the Corium Consulting Agreement, we received payments from Corium of $15.6 million, $13.6 million of which was paid in quarterly installments through March 31, 2022. The remaining $2.0 million was received in the first quarter of 2022 upon the approval by the FDA of the NDA for Corium's product known as ADLARITY.

We have had recurring negative net operating cash flows and we anticipate that we may continue to incur minimal positive net cash flows from operations or negative net cash flows from operations for at least the next several years. We expect that our sources of revenue will be through payments arising from our license agreement with Corium, or through our Corium Consulting Agreement, and other potential consulting arrangements and any other future arrangements related to one of our product candidates

We filed a registration statement on Form S-3 covering the sale of the shares of our common stock up to $350.0 million, $75.0 million of which was allocated to the sales of the shares of common stock issuable under the Equity Distribution Agreement. The Form S-3 was declared effective on July 12, 2021. As of December 31, 2023, no shares have been issued or sold under the Equity Distribution Agreement.

We have incurred operating losses since our inception and, as of December 31, 2023, had an accumulated deficit of $399.8 million. We anticipate that we will continue to incur operating losses for at least the next several years. Our recurring operating losses and negative cash flows from operations raise substantial doubt about our ability to continue as a going concern. We have currently have insufficient sources of revenues to sustain our present activities. Accordingly, our ability to continue as a going concern will require us to obtain additional financing to fund our operations. The perception of our inability to continue as a going concern may make it more difficult for us to obtain financing for the continuation of our operations and could result in the loss of confidence by investors, suppliers and employees. Adequate additional financing may not be available to us on acceptable terms, or at all. To the extent that we raise additional capital through the sale of equity or debt, the terms of these securities may restrict our ability to operate. If we raise additional funds through collaborations, strategic alliances or marketing, distribution or licensing arrangements with third parties, we may be required to relinquish valuable rights. If we are unable to raise capital when needed or on attractive terms, we could be forced to delay, reduce or altogether cease our research and development programs or future commercialization efforts.

Equity Distribution Agreement

On July 2, 2021, we entered into an Equity Distribution Agreement with JMP and RBCCM, under which we may offer and sell, from time to time at our sole discretion, shares of our common stock having an aggregate offering price of up to $75.0 million through JMP and RBCCM as our sales agents. The issuance and sale, if any, of our common stock under the Equity Distribution Agreement will be made pursuant to a registration statement on Form S-3.

Share Repurchase Program

On December 20, 2021, we initiated the Share Repurchase Program pursuant to which we may repurchase up to $50 million of shares of its common stock through December 31, 2023. On December 31, 2023, the Share Repurchase Program ended, and we have repurchased 1,575,692 shares of its common stock for approximately $11.0 million.

Merger Transactions and Documents

On August 30, 2023, in connection with the Merger Agreement with Acer, the following transactions occurred prior to Closing:

- *Bridge Loan* - Zevra and Acer entered into a bridge loan agreement (the "Bridge Loan Agreement"), providing for Zevra to make loans (collectively, the "Bridge Loan") to Acer up to an aggregate principal amount of $16.5 million. The Bridge Loan was provided to Acer to support its termination agreement with Relief Therapeutics Holding SA ("Relief") and to provide Acer with working capital, including for payments of accounts payable to support the commercial launch of OLPRUVA and the development of celiprolol pending the Merger's closure. On October 31, 2023, the Company and Acer entered into an amendment to the Bridge Loan Agreement, which increased the aggregate principal amount available under the loan from $16.5 million to $17.8 million.

- *Purchase of Acer's Term Loans* - Zevra purchased certain indebtedness of Acer held by Nantahala Capital Management, LLC ("Nantahala). Under the loan purchase with Nantahala, certain of its affiliates and certain other parties (collectively with Nantahala, "Nantahala Holders") Zevra purchased (i) an original senior secured term loan facility made available to Acer in an aggregate amount of $6.5 million and funded on March 14, 2022, and (ii) an additional senior secured term loan made to Acer in an aggregate amount of $7.0 million in a single borrowing which funded on January 31, 2023 for (1) $12.0 million in cash; (2) 98,683 shares of Zevra Common Stock; and (3) a secured Promissory Note payable by Zevra to Nantahala in the original principal amount $5.0 million. These were recorded as receivables form Acer and were treated as a settlement of a preexisting relationship in connection with the closing of the transaction and recorded as a component of purchase consideration.

- *Purchase of Acer's Convertible* Notes ("Marathon Convertible Notes")- Under the Note Purchase Agreement with the Nantahala Holders, Zevra purchased the Marathon Convertible Notes that Nantahala had acquired on June 16, 2023. Zevra acquired the Marathon Convertible Notes in exchange for the issuance of 2,171,038 shares of Zevra Common Stock at $5.0667 per share for a total purchase price of $11.0 million.

- *Amendment to IP License Agreement and IP Termination Agreement:* As a condition to entering into the Merger Agreement, Acer and Relief Therapeutics Holding AG ("Relief") entered into the Exclusive License Agreement and the Termination Agreement terminating the collaboration and license agreement, dated March 19, 2021, by and between Acer and Relief. Pursuant to the Exclusive License Agreement, Relief holds exclusive development and commercialization rights for OLPRUVA in the European Union, Liechtenstein, San Marino, Vatican City, Norway, Iceland, Principality of Monaco, Andorra, Gibraltar, Switzerland, United Kingdom, Albania, Bosnia, Kosovo, Montenegro, Serbia and North Macedonia (Geographical Europe). Acer has the right to receive a royalty of up to 10.0% of the net sales of OLPRUVA in Geographical Europe. In accordance with the terms of the Termination Agreement, Relief received an upfront payment from Acer of $10.0 million (which payment was funded with the Bridge Loan described above) with an additional payment of $1.5 million due on the first-year anniversary of the $10.0 million payment. Acer has also agreed to pay a 10.0% royalty on net sales of OLPRUVA worldwide, excluding Geographical Europe, and 20.0% of any value received by Acer from certain third parties relating to OLPRUVA licensing or divestment rights, all of the foregoing which are capped at $45.0 million, for total payments to Relief of up to $56.5 million.

In connection with the closing of the Merger on November 17, 2023, each share of common stock of Acer was converted into the right to receive (i) 0.1210 fully paid and non-assessable shares of common stock of Zevra, par value $0.0001 per share , and (ii) one non-transferable contingent value right ("CVR") to be issued by Zevra, which will represent the right to receive one or more contingent payments up to an additional $76 million upon the achievement, if any, of certain commercial and regulatory milestones for Acer's OLPRUVA and celiprolol products within specified time periods. Certain additional cash payments are also possible pursuant to the CVRs with respect to milestones involving Acer's early-stage program ACER-2820 (emetine).

Registration Rights Agreement

Zevra and Nantahala concurrently entered into a registration rights agreement (the "Registration Rights Agreement"), pursuant to which Zevra agreed to file a resale registration statement with respect to the resale of the Zevra common stock issuable under the Loan and Note Purchase Agreements and the Nantahala Note. On February 5, 2024, Zevra filed a registration statement on Form S-3 (File No. 333-276856) registering an aggregate of 2,269,721 shares of Zevra's common stock that were issued pursuant to the Loan and Note Purchase Agreements.

Stockholders Agreement

In connection with of the Merger, a certain stockholder of Acer entered into, and Acer agreed to use its reasonable best efforts to cause certain other stockholders to enter into joinders to, a stockholders agreement with Zevra (the "Stockholders Agreement"). Pursuant to the Stockholders Agreement, the stockholders party thereto agreed to, or would agree to, among other things, vote all of their shares in Zevra that they own in favor of each nominee included in the Zevra board of director's slate of nominees for each election of directors and in favor of each matter approved by the Zevra board of directors and submitted to stockholders of Zevra for the approval of stockholders following the closing of the Merger and until the second anniversary of the closing date of the Merger (the "Trigger Date"). In addition, the stockholders party to the Stockholders Agreement will be subject to customary standstill provisions, subject to certain exceptions, until the Trigger Date.

Cancellation of Acer Warrant

On November 22, 2023, we sold an aggregate of 1,382,489 shares of our common stock and accompanying warrants to purchase up to 1,382,489 shares of our common stock at a price of $4.34 per share to a healthcare focused investment fund (the "Investor") for gross proceeds of approximately $6.0 million and an aggregate of 917,934 shares of our common stock to cancel a warrant held by the Investor to purchase 2,920,306 shares of common stock of Acer. The shares of common stock and warrants were offered and sold to the Investor in a registered direct offering without an underwriter or placement agent.

Line of Credit

On May 31, 2022, we and Ameris Bank, as lender, entered into a $20.0 million revolving loan agreement, or the Line of Credit. Proceeds of the revolving facility provided by the Line of Credit are to be used for general corporate purposes. Loans under the Line of Credit bear interest at the Secured Overnight Financing Rate, or the SOFR, plus 1.60%, with a SOFR floor of 0.00%.

The revolving facility under the Line of Credit is secured by a perfected security interest in deposit accounts. The revolving facility under the Line of Credit is subject to customary affirmative and negative covenants.

The latest maturity date of the loans under the Line of Credit is May 31, 2025. The Line of Credit contains customary events of default that could lead to an acceleration of the loans, including cross-default, bankruptcy and payment defaults. As of December 31, 2022, we had drawn $12.8 million from the Line of Credit to finance the transactions under the Arimoclomol Purchase Agreement, and this amount is supported by a $12.8 million certificate of deposit which is shown as long-term investments -other in the consolidated balance sheet as of December 31, 2022. The remaining $7.2 million under the Line of Credit was secured by a separate interest-bearing certificate of deposit and was also recorded as long-term investments - other in the consolidated balance sheet as of December 31, 2022. These certificates of deposit are pledged as collateral against the Line of Credit and cannot be redeemed so long as the $20.0 million remains available under the Line of Credit. The total value of the certificates of deposit held with Ameris Bank must meet or exceed the amount available to borrow under the Line of Credit so long as the Line of Credit remains active. On January 31, 2023, we repaid the $12.8 million outstanding under the Line of Credit in full, and subsequently closed the Line of Credit during the first quarter of 2023. In conjunction with closing the Line of Credit, the maturity dates of the certificates of deposit were modified to May 2023.

On January 26, 2023, the Company and Wells Fargo, as lender, entered into a margin account agreement. The margin account bears interest at the Prime Rate minus 225 basis points. The Company's investments are used as collateral for the loan and the amount the Company is able to borrow is limited to 80-90% of its outstanding investment balance held with Wells Fargo. As of December 31, 2023, $37.7 million was outstanding under the margin account.

Cash Flows

The following table summarizes our cash flows for the years ended December 31, 2023, and 2022 (in thousands):

	Year Ended December 31,		Period-to Period Change
	2023	2022	
Net cash used in operating activities	$ (33,535)	$ (18,717)	$ (14,818)
Net cash used in investing activities	(17,390)	(36,719)	19,329
Net cash provided by financing activities	28,464	8,352	20,112
Effect of exchange rate changes on cash and cash equivalents	44	204	(160)
Net decrease in cash and cash equivalents	$ (22,417)	$ (46,880)	$ 24,463

Operating Activities

For the year ended December 31, 2023, net cash used in operating activities of $33.5 million consisted of a net loss of $46.0 million, partially offset by $6.0 million in changes in working capital and $6.5 million in adjustments for non-cash items. Net loss was primarily attributable to our spending on research and development programs and operating costs, partially offset by revenue received under the AZSTARYS License Agreement, Arimoclomol EAP and the Corium Consulting Agreement. The changes in working capital consisted of $11.1 million related to a change in accounts payable and accrued expenses, $3.2 million related to a change in discount and rebate liabilities, $0.2 million related to a change in inventories, $0.3 million related to a change in operating lease right of use assets, $0.4 million related to a change in other liabilities, and $0.2 million related to a change in prepaids and other assets, partially offset by a $9.1 million increase in accounts and other receivables, and $0.3 million related to operating lease liabilities. The adjustments for non-cash items primarily consisted of stock-based compensation expense of $5.9 million, consulting fees paid in stock of $0.2 million, and $1.0 million related to depreciation, amortization and other items, partially offset by a change in the fair value adjustment related to investments of $0.6 million.

For the year ended December 31, 2022, net cash used in operating activities of $18.7 million consisted of a net loss of $41.5 million and $0.5 million in changes in working capital, partially offset by $23.3 million in adjustments for non-cash items. Net loss was primarily attributable to our spending on research and development programs and operating costs, partially offset by revenue received under the AZSTARYS License Agreement, Arimoclomol EAP and the Corium Consulting Agreement. The changes in working capital consisted of $0.7 million related to a change in prepaid expenses and other assets, $6.8 million related to a change in accounts and other receivables, $0.4 million related to a change in operating lease liabilities and $0.4 million related to a change in other liabilities, partially offset by $3.1 million related to a change in accounts payable and accrued expenses, $0.1 million related to a change in inventories, $0.3 million related to a change in operating lease right-of-use assets, $0.4 million related to other long-term assets, and $3.8 million related to a change in discount and rebate liabilities. The adjustments for non-cash items primarily consisted of stock-based compensation expense of $4.3 million, consulting fees paid in stock of $0.2 million, a change in the fair value adjustment related to investments of $0.6 million, $17.7 million related to acquired in-process research and development which was expensed as part of the transactions under the Arimoclomol Purchase Agreement and $0.9 million related to depreciation, amortization and other items partially offset by a change in the fair value adjustment related to derivative and warrant liabilities of $0.3 million.

Investing Activities

For the year ended December 31, 2023, net cash used in investing activities was $17.4 million, which was attributable to $30.4 million attributable to the acquisition of Acer, purchases of investments of $45.8 million, and $0.3 million in purchases of property and equipment, partially offset by maturities of investments of $59.1 million.

For the year ended December 31, 2022, net cash used in investing activities was $36.7 million, which was attributable to net acquisition costs of the transactions under the Arimoclomol Purchase Agreement of $14.1 million and purchases of investments of $23.8 million, partially offset by maturities of investments of $1.3 million.

Financing Activities

For the year ended December 31, 2023, net cash provided by financing activities was $28.5 million, which was primarily attributable to proceeds from the issuance of debt of $42.4 million, proceeds from insurance financing arrangements of $1.3 million and proceeds from sales of common stock under the Employee Stock Purchase Plan, or the ESPP, of $0.2 million, proceeds from issuance of common stock of $6.0 million, partially offset by repayments of debt of $17.5 million, payments to repurchase shares as part of the Share Repurchase Program of $3.4 million, and payments of principal on insurance financing arrangements of $0.5 million.

For the year ended December 31, 2022, net cash provided by financing activities was $8.3 million, which was primarily attributable to proceeds from the issuance of debt of $12.8 million, proceeds from insurance financing arrangements of $1.3 million and proceeds from sales of common stock under the Employee Stock Purchase Plan, or the ESPP, of $0.3 million, partially offset by payments to repurchase shares as part of the Share Repurchase Program of $4.7 million, and payments of principal on insurance financing arrangements of $1.3 million.

Future Funding Requirements

The auditor's opinion on our audited financial statements for the year ended December 31, 2023, includes an explanatory paragraph stating that our recurring losses, negative operating cash flows and stockholders' deficit raise substantial doubt about our ability to continue as a going concern. We currently have insufficient sources of revenues to sustain our present activities. Accordingly, our ability to continue as a going concern will require us to obtain additional financing to fund our operations. The perception of our inability to continue as a going concern may make it more difficult for us to obtain financing for the continuation of our operations and could result in the loss of confidence by investors, suppliers and employees. Adequate additional financing may not be available to us on acceptable terms, or at all. To the extent that we raise additional capital through the sale of equity or debt, the terms of these securities may restrict our ability to operate. If we raise additional funds through collaborations, strategic alliances or marketing, distribution or licensing arrangements with third parties, we may be required to relinquish valuable rights. If we are unable to raise capital when needed or on attractive terms, we could be forced to delay, reduce or altogether cease our research and development programs or commercialization efforts.

Based on our current operating forecast, we believe that our existing cash, cash equivalents and long-term investments will be sufficient to fund our operations into, but not through, 2026. Based on our current operating plan, our existing resources are expected to be sufficient to fund operating expenses and capital investment requirements into, but not through, 2026. However, unless we are able to restructure the amounts outstanding on the margin loan facility, we may be required to repay the loan and thereby deplete the cash available to fund our operations.

Potential near-term sources of additional funding include:

- any royalties or net sales milestone payments generated under the AZSTARYS License Agreement;
- any product sales under the Arimoclomol EAP;
- any product sales of OLPRUVA
- any product sales of arimoclomol, if approved; and
- any consulting services revenue generated under other potential consulting arrangements.

We cannot guarantee that we will be able to generate sufficient proceeds from any of these potential sources to fund our operating expenses.

To date, we have generated revenue from the AZSTARYS License Agreement, reimbursements of out-of-pocket third-party costs, the performance of consulting services, OLPRUVA product sales, and product sales under the Arimoclomol EAP. We expect that, for the foreseeable future, our only sources of revenues will be through payments arising from the AZSTARYS License Agreement, product sales of OLPRUVA. through potential consulting arrangements and any other future arrangements related to one of our product candidates and product sales under the Arimoclomol EAP. While we have entered into the AZSTARYS License Agreement to develop, manufacture and commercialize AZSTARYS, we cannot guarantee that this, or any strategy we adopt in the future, will be successful. For instance, we received milestone payments under the AZSTARYS License Agreement, but we cannot guarantee that we will earn any additional milestone or royalty payments under this agreement in the future. We also cannot guarantee that we will continue to generate revenue under the Arimoclomol EAP or successfully commercialize OLPRUVA. We also expect to continue to incur additional costs associated with operating as a public company.

We have based our estimates of our cash needs and cash runway on assumptions that may prove to be wrong, and we may use our available capital resources sooner than we currently expect and we cannot guarantee that we will be able to generate sufficient proceeds from the AZSTARYS License Agreement, product reimbursements under the Arimoclomol EAP, product sales of OLPRUVA, potential consulting arrangements or other funding transactions to fund our operating expenses. To meet any additional cash requirements, we may seek to sell additional equity or convertible securities that may result in dilution to our stockholders, issue additional debt or seek other third-party funding, including potential strategic transactions, such as licensing or collaboration arrangements. Because of the numerous risks and uncertainties associated with the development and commercialization of product candidates and products, we are unable to estimate the amounts of increased capital outlays and operating expenditures necessary to complete the commercialization and development of our partnered product or product candidates, should they obtain regulatory approval.

Critical Accounting Estimates

This management's discussion and analysis of our financial condition and results of operations is based on our financial statements, which we have prepared in accordance with accounting principles generally accepted in the United States. The preparation of our financial statements requires us to make estimates that affect the reported amounts of assets and liabilities and the disclosure of contingent assets and liabilities at the date of our financial statements, as well as the reported revenues and expenses during the reported periods. We evaluate these estimates on an ongoing basis. We base our estimates on historical experience and on various other factors that we believe are reasonable under the circumstances. Actual results may differ from these estimates under different assumptions or conditions.

While our significant accounting policies are more fully described in Note B to our audited financial statements appearing elsewhere in this Annual Report on Form 10-K, we believe that the following accounting policies are critical to the process of making significant estimates in the preparation of our financial statements and understanding and evaluating our reported financial results.

Acquisition of Intangible Assets

We record all assets and liabilities acquired in business acquisitions at fair value, including goodwill and other intangible assets. The initial recognition of goodwill and other intangible assets requires management to make subjective judgments concerning estimates of how the acquired assets will perform in the future using valuation methods including discounted cash flow analysis. Inherent in the determination of fair value of the reporting units are certain estimates and judgments, including the interpretation of current economic indicators and market valuations, as well as management's strategic plans with regard to its operations. When utilizing a quantitative assessment, we determine fair value at the reporting unit level based on a combination of an income approach and market approach. The income approach is based on estimated future cash flows, discounted at a rate that approximates the cost of capital of a market participant, while the market approach is based on sales and/or earnings multiples of similar companies. These approaches use significant estimates and assumptions, including projected future cash flows and the timing of those cash flows, discount rates reflecting risks inherent in future cash flows, perpetual growth rates, and determination of appropriate market comparables.

We accounted for the arimoclomol acquisition as an asset acquisition as the majority of the value of the assets acquired related to the arimoclomol acquired in-process research and development, or the IPR&D asset. The intangible asset associated with IPR&D relates to arimoclomol. The estimated fair value of $17.7 million was determined using the excess earnings valuation method, a variation of the income valuation approach. The excess earnings valuation method estimates the value of an intangible asset equal to the present value of the incremental after-tax cash flows attributable to that intangible asset over its remaining economic life. Some of the more significant assumptions utilized in our asset valuations included projected revenues, probability of commercial success, and the discount rate. The fair value using the excess earnings valuation method was determined using an estimated weighted average cost of capital of 42%, which reflects the risks inherent in future cash flow projections and represents a rate of return that a market participant would expect for this asset. This fair value measurement was based on significant inputs not observable in the market and thus represent Level 3 fair value measurement.

Goodwill and Definite-lived Intangible Assets

Goodwill represents the excess of the purchase price of an acquired business over the fair value assigned to the assets purchased and liabilities assumed. Goodwill is not amortized but is evaluated for impairment within our single reporting unit on an annual basis, during the fourth quarter, or more frequently if an event occurs or circumstances change that would more-likely-than-not reduce the fair value of our reporting unit below its carrying amount. Our estimates associated with the annual test of goodwill for impairment, as well as the as-needed assessment of the recoverability of definite-lived intangible assets, are considered critical due to the amount of these assets recorded on our consolidated balance sheets and the judgment required.

With respect to definite-lived intangible assets, we periodically evaluate whether events and circumstances have occurred that may affect the estimated useful life or the recoverability of the remaining balance of such assets. If such events or circumstances indicate that the carrying amount of these assets may not be recoverable, management would estimate the future cash flows expected to result from the use of the assets and their eventual disposition. If the sum of the expected future cash flows (undiscounted and without interest charges) were less than the carrying amount of the assets, we would recognize an impairment charge to reduce such assets to their fair value.

Accrued Expenses

We enter into contractual agreements with third-party vendors who provide research and development, manufacturing, and other services in the ordinary course of business. Some of these contracts are subject to milestone-based invoicing and services are completed over an extended period of time. We record liabilities under these contractual commitments when an obligation has been incurred. This accrual process involves reviewing open contracts and purchase orders, communicating with our applicable personnel to identify services that have been performed and estimating the level of service performed and the associated cost when we have not yet been invoiced or otherwise notified of actual cost. The majority of our service providers invoice us monthly in arrears for services performed. We make estimates of our accrued expenses as of each balance sheet date based on the facts and circumstances known to us. We periodically confirm the accuracy of our estimates with the service providers and make adjustments if necessary. Examples of estimated accrued expenses include:

- fees paid to CROs in connection with preclinical and toxicology studies and clinical trials;
- fees paid to investigative sites in connection with clinical trials;
- fees paid to contract manufacturers in connection with the production of our raw materials, drug substance and product candidates; and
- professional fees.

We base our expenses related to clinical trials on our estimates of the services received and efforts expended pursuant to contracts with multiple research institutions and CROs that conduct and manage clinical trials on our behalf. The financial terms of these agreements are subject to negotiation, vary from contract to contract and may result in uneven payment flows. Payments under some of these contracts depend on factors such as the successful enrollment of patients and the completion of clinical trial milestones. In accruing service fees, we estimate the time period over which services will be performed and the level of effort expended in each period. If the actual timing of the performance of services or the level of effort varies from our estimate, we will adjust the accrual accordingly. If we do not identify costs that we have begun to incur or if we underestimate or overestimate the level of these costs, our actual expenses could differ from our estimates.

Stock-Based Compensation

We record the fair value of stock options issued as of the grant date as compensation expense. We recognize compensation expense over the requisite service period, which is equal to the vesting period. Stock-based compensation expense has been reported in our statements of operations as follows (in thousands):

	Year Ended December 31,	
	2023	2022
Research and development	$ 2,664	$ 1,443
General and administrative	3,290	2,851
Total stock-based compensation	$ 5,954	$ 4,294

Determination of the Fair Value of Stock-Based Compensation Grants

We calculate the fair value of stock-based compensation arrangements using the Black-Scholes option-pricing model. The Black-Scholes option-pricing model requires the use of subjective assumptions, including the expected volatility of our common stock, the assumed dividend yield, the expected term of our stock options, the risk-free interest rate for a period that approximates the expected term of our stock options and the fair value of the underlying common stock on the date of grant. In applying these assumptions, we considered the following factors:

- historically we have not had sufficient experience to estimate the volatility of our common stock. As such, we calculated the expected volatility based on reported data for selected similar publicly traded companies for which the historical information is available, or peer volatility, and blended it with our historical volatility, or leverage-adjusted peer volatility. For the purpose of identifying peer companies, we consider characteristics such as industry, length of trading history, similar vesting terms and in-the-money option status. We utilized this leverage-adjusted peer volatility for grants prior to the initial public offering, as well as grants within the two-year period immediately following the initial public offering. For grants after the second anniversary of the initial public offering we utilized our historical volatility to determine the expected volatility;

- the assumed dividend yield is based on our expectation of not paying dividends for the foreseeable future;

- we determine the average expected life of “plain vanilla” stock options based on the simplified method in accordance with SEC Staff Accounting Bulletin Nos. 107 and 110, as our common stock to date has been publicly traded for a limited amount of time. We expect to use the simplified method until we have sufficient historical exercise data to provide a reasonable basis upon which to estimate expected term. For options that are not considered “plain vanilla,” such as those with exercise prices in excess of the fair market value of the underlying stock, we use an expected life equal to the contractual term of the option;

- we determine the risk-free interest rate by reference to implied yields available from U.S. Treasury securities with a remaining term equal to the expected life assumed at the date of grant; and

- we estimate forfeitures based on our historical analysis of actual stock option forfeitures.

We account for stock-based compensation arrangements with directors and consultants that contain only service conditions for vesting using a fair value approach. The grant date fair value of these options is measured using the Black-Scholes option pricing model reflecting the same assumptions as applied to employee options in each of the reported periods, other than the expected life, which is assumed to be the remaining contractual life of the option.

The following summarizes the assumptions used for estimating the fair value of stock options granted to employees for the periods indicated:

	Year Ended December 31,	
	2023	2022
Risk-free interest rate	3.34% - 4.79%	1.70% - 3.80%
Expected term (in years)	5.50 - 10.00	5.50 - 7.00
Expected volatility	89.48% - 93.67%	91.28% - 98.91%
Expected dividend yield	0	0

Utilization of Net Operating Loss Carryforwards and Research and Development Credits

As of December 31, 2023, we had federal net operating loss, or NOL, carryforwards of approximately $350 million, $145.3 million of which, if not utilized, will begin to expire in 2027 and $204.7 million of which have no expiration date. We also have certain state net operating loss carryforwards totaling $340 million, which, if not utilized, will begin to expire in 2027. We also have Denmark net operating loss carryforwards totaling $4.7 million which have an indefinite carryforward period in Denmark. The Company recorded refundable research and development tax credit as other income and not income tax under ASC 740 in the consolidated statement of operations for the year ended December 31, 2023. These refundable tax credits are a result of increased qualified research and development spending in certain jurisdictions which allow for a refundable credit even when the Company has no current period income tax expense.

In accordance with Section 382 of the Code, a change in equity ownership of greater than 50% within a three-year period results in an annual limitation on a company’s ability to utilize its NOL carryforwards created during the tax periods prior to the change in ownership. We performed a Section 382 ownership change analysis in 2017 and determined that we experienced an ownership change in 2010, which resulted in a portion of our net operating loss carryforwards being subject to an annual limitation under Section 382 through 2012. No other ownership changes or limitations on our historical net operating loss carryforwards were noted through the year ended December 31, 2017. In addition, we may have already experienced an ownership change, or may experience an ownership changes in the future, as a result of shifts in our stock ownership. If we determine that an ownership change has occurred and our ability to use our historical net operating loss carryforwards is materially limited, it would harm our future operating results by increasing our future tax obligations.

Warrants

We account for warrants as either equity-classified or liability-classified instruments based on an assessment of the warrant's specific terms and applicable authoritative guidance in FASB ASC Topic 480, Distinguishing Liabilities from Equity (ASC 480) and FASB ASC Topic 815, Derivatives and Hedging (ASC 815). The assessment considers whether the warrants are freestanding financial instruments pursuant to ASC 480, meet the definition of a liability pursuant to ASC 480, and whether the warrants meet all of the requirements for equity classification under ASC 815, including whether the warrants are indexed to our own stock and whether the warrant holders could potentially require "net cash settlement" in a circumstance outside of the company's control, among other conditions for equity classification. This assessment, which requires the use of professional judgment, is conducted at the time of warrant issuance and as of each subsequent quarterly period end date while the warrants are outstanding.

For warrants that meet all criteria for equity classification, the warrants are required to be recorded as a component of additional paid-in capital, on the consolidated statement of stockholders' deficit at the time of issuance. For warrants that do not meet all the criteria for equity classification, the warrants are required to be recorded at their initial fair value on the date of issuance, and on each balance sheet date thereafter. Changes in the estimated fair value of the warrants are recognized as a non-cash gain or loss in other expense, net, on the consolidated statement of operations. The fair value of the warrants was estimated using the Black-Scholes option pricing model.

In connection with the preparation of this Annual Report on Form 10-K for the fiscal year ended December 31, 2023, the Audit Committee concluded that, in prior periods it had not appropriately accounted for certain common stock warrants as liabilities. These errors led to understatements of derivative and warrant liability and additional paid-in capital and fluctuations in fair value adjustment related to derivative and warrant liability during the impacted periods. See "Explanatory Note."

ITEM 7A. QUANTITATIVE AND QUALITATIVE DISCLOSURES ABOUT MARKET RISK

Not applicable.

ITEM 8. FINANCIAL STATEMENTS AND SUPPLEMENTARY DATA

The financial statements required by this Item 8 are set forth beginning in Item 15 of this report and are incorporated herein by reference.

ITEM 9. CHANGES IN AND DISAGREEMENTS WITH ACCOUNTANTS ON ACCOUNTING AND FINANCIAL DISCLOSURE

None.

ITEM 9A. CONTROLS AND PROCEDURES

Limitations on effectiveness of controls and procedures

In designing and evaluating our disclosure controls and procedures, management recognizes that any controls and procedures, no matter how well designed and operated, can provide only reasonable assurance of achieving the desired control objectives. In addition, the design of disclosure controls and procedures must reflect the fact that there are resource constraints and that management is required to apply judgment in evaluating the benefits of possible controls and procedures relative to their costs.

Evaluation of Disclosure Controls and Procedures

Our management, with the participation of our chief executive officer and our chief financial officer, evaluated the effectiveness of our disclosure controls and procedures as of December 31 2023. During year-end control procedures, management identified a material weakness in our internal control over financial reporting related to the accounting for warrants to purchase the Company's common stock. The material weakness existed as of December 31, 2023, and prior periods. The nature of the material weakness is described as part of Management's Report on our Internal Control over Financial Reporting included below.

As a result of the material weakness identified in Management's Report on Internal Control over Financial Reporting related to accounting for warrants as discussed below, our Chief Executive Officer and Chief Financial Officer have concluded that our disclosure controls and procedures were not effective at a reasonable assurance level as of December 31, 2023.

Management's Report on Internal Control over Financial Reporting

Management is responsible for establishing and maintaining adequate internal control over our financial reporting, as such term is defined in Rules 13a-15(f) and 15d-15(e) under the Exchange Act. Under the supervision and with the participation of our management, including our chief executive officer and chief financial officer, we conducted an evaluation of the effectiveness of our internal control over financial reporting. Management has used the framework set forth in the report entitled "*Internal Control – Integrated Framework (2013)"* published by the Committee of Sponsoring Organizations of the Treadway Commission to evaluate the effectiveness of our internal control over financial reporting. Our evaluation of internal control over financial reporting did not include internal controls of Acer, a wholly-owned subsidiary acquired in November 2023. This fiscal year 2023 acquisition represented 49.3% of our consolidated total assets and 0.2% of our consolidated total revenue as of and for the fiscal year ended December 31, 2023. We have included the financial results of the acquired operations in our consolidated financial statements included elsewhere in this Annual Report on Form 10-K from the date of acquisition, and we are in the process of incorporating Acer Therapeutics Inc. into our internal control over financial reporting.

Material Weakness

A material weakness is a deficiency, or combination of deficiencies, in internal control over financial reporting such that there is a reasonable possibility that a material misstatement of the Company's annual or interim financial statements will not be prevented or detected on a timely basis. The material weakness in our internal control resulted in the restatement of the Company's consolidated financial statements as of and for the year ended December 31, 2022, and the condensed consolidated financial statements for the interim periods ended March 31, 2022, June 30, 2022, September 30, 2022, March 31, 2023, June 30, 2023, and September 30, 2023, included in this report. As a result of the material weakness noted above, management has concluded that our internal control over financial reporting was not effective as of December 31, 2023. Ernst & Young LLP, an independent registered public accounting firm, has audited the consolidated financial statements included in this Annual Report on Form 10-K and, has not issued an attestation report on the effectiveness of our internal control over financial reporting. The material weakness described above, which related to internal controls over the application of specific technical accounting guidance, was identified during the performance of our year-end control procedures.

Our independent registered public accounting firm has not performed an evaluation of our internal control over financial reporting during any period in accordance with the provisions of the Sarbanes-Oxley Act. For as long as we remain a non-accelerated filer, we intend to take advantage of the exemption permitting us not to comply with the requirement that our independent registered public accounting firm provide an attestation on the effectiveness of our internal control over financial reporting.

Remediation of Material Weakness

We have implemented certain remedial measures including, but not limited to, a review of all existing accounting for warrants to purchase the Company' s common stock to confirm compliance with GAAP prior to filing the consolidated financial statements as of and for the year ended December 31, 2023 with this Annual Report on Form 10-K.

In addition, we are in the process of developing enhanced control procedures designed to ensure proper accounting for our warrant related accounts and balances, which will include adding technical resources to perform and oversee technical accounting. Based on additional procedures and post-closing review, management has concluded that the consolidated financial statements included in this Annual Report on Form 10-K present fairly, in all material respects, our financial position, results of operations, and cash flows for the periods presented, in conformity with GAAP.

However, the material weakness cannot be considered remediated until the applicable remedial controls operate for a sufficient period of time and management has concluded, through testing that these controls are operating effectively.

Changes in Internal Control over Financial Reporting

Except as disclosed above, there was no change in our internal control over financial reporting identified in connection with the evaluation required by Rules 13a-15(d) and 15d-15(d) of the Exchange Act that occurred during our fiscal quarter ended December 31, 2023, that materially affected, or is reasonably likely to materially affect, our internal control over financial reporting.

ITEM 9B. OTHER INFORMATION

Trading Arrangements

During the three-months ended December 31, 2023, none of our directors or officers (as defined in Rule 16a-1(f) of the Exchange Act) adopted, modified or terminated any contract, instruction or written plan for the purchase or sale of our securities that was intended to satisfy the affirmative defense conditions of Rule 10b5-1(c) of the Exchange Act or any non-Rule 10b5-1 trading arrangement (as defined in Item 408(c) of Regulation S-K).

ITEM 9C. DISCLOSURES REGARDING FOREIGN JURISDICTIONS THAT PREVENT INSPECTIONS

Not applicable.

PART III

ITEM 10. DIRECTORS, EXECUTIVE OFFICERS AND CORPORATE GOVERNANCE

The information required by this Item 10 will be set forth in the headings “Proposal 1 – Election of Directors,” “Executive Officers” and “Information Regarding the Board of Directors and Corporate Governance” and "Delinquent Section 16(a) Reports" in our definitive proxy statement for our 2024 annual meeting of stockholders, or the proxy statement, and, is incorporated herein by reference.

We have adopted a Code of Business Conduct and Ethics, or the Code of Conduct, applicable to all of our employees, executive officers and directors, including our principal executive officer, principal financial officer, principal accounting officer or controller, or persons performing similar functions. The Code of Conduct is available on our website at www.zevra.com. The nominating and corporate governance committee of our board of directors is responsible for overseeing the Code of Conduct and must approve any waivers of the Code of Conduct for employees, executive officers and directors. We intend to post any amendments to the Code of Conduct or any waivers of its requirements for any executive officer or director on our website.

ITEM 11. EXECUTIVE COMPENSATION

The information required by this Item 11 will be set forth under the headings “Executive Compensation", "Director Compensation" and “Information Regarding the Board of Directors and Corporate Governance” in our proxy statement and is incorporated herein by reference.

ITEM 12. SECURITY OWNERSHIP OF CERTAIN BENEFICIAL OWNERS AND MANAGEMENT

The information required by this Item 12 will be set forth under the headings “Security Ownership of Certain Beneficial Owners and Management” and “Securities Authorized for Issuance under the Equity Compensation Plans” in the proxy statement and is incorporated herein by reference.

ITEM 13. CERTAIN RELATIONSHIPS AND RELATED TRANSACTIONS, AND DIRECTOR INDEPENDENCE

The items required by this Item 13 will be set forth under the headings “Information Regarding the Board of Directors and Corporate Governance” and “Transactions with Related Persons” in the proxy statement and is incorporated herein by reference.

ITEM 14. PRINCIPAL ACCOUNTING FEES AND SERVICES

The information required by this Item 14 will be set forth under the proposal with the heading “Proposal 2 - Ratification of Appointment of Independent Registered Public Accounting Firm” in the proxy statement and is incorporated herein by reference.

PART IV

ITEM 15. EXHIBITS AND FINANCIAL STATEMENT SCHEDULES

(a) The following documents are filed as part of this report:

(1) *Index list to Financial Statements:*

(2) *Financial Statement Schedules*

All other schedules are omitted because they are not required or the required information is included in the financial statements or notes thereto.

(3) *Exhibits*

The exhibits listed in the accompanying Exhibit Index are filed or incorporated by reference as part of this report.

Report of Independent Registered Public Accounting Firm

To the Stockholders and the Board of Directors of Zevra Therapeutics, Inc.

Opinion on the Financial Statements

We have audited the accompanying consolidated balance sheets of Zevra Therapeutics, Inc. (the Company) as of December 31, 2023 and 2022, the related consolidated statements of operations, comprehensive income (loss), changes in stockholders' equity and cash flows for each of the two years in the period ended December 31, 2023, and the related notes (collectively referred to as the "consolidated financial statements"). In our opinion, the consolidated financial statements present fairly, in all material respects, the financial position of the Company at December 31, 2023 and 2022, and the results of its operations and its cash flows for each of the two years in the period ended December 31, 2023, in conformity with U.S. generally accepted accounting principles.

Restatement of 2022 Financial Statements

As discussed in Note C to the consolidated financial statements, the financial statements as of and for the year ended December 31, 2022 have been restated to correct misstatements.

The Company's Ability to Continue as a Going Concern

The accompanying consolidated financial statements have been prepared assuming that the Company will continue as a going concern. As discussed in Note A to the consolidated financial statements, the Company has sustained recurring losses and negative cash flows from operations, and has stated that substantial doubt exists about the Company's ability to continue as a going concern. Management's evaluation of the events and conditions and management's plans regarding these matters are also described in Note A. The consolidated financial statements do not include any adjustments that might result from the outcome of this uncertainty.

Basis for Opinion

These financial statements are the responsibility of the Company's management. Our responsibility is to express an opinion on the Company's financial statements based on our audits. We are a public accounting firm registered with the Public Company Accounting Oversight Board (United States) (PCAOB) and are required to be independent with respect to the Company in accordance with the U.S. federal securities laws and the applicable rules and regulations of the Securities and Exchange Commission and the PCAOB.

We conducted our audits in accordance with the standards of the PCAOB. Those standards require that we plan and perform the audit to obtain reasonable assurance about whether the financial statements are free of material misstatement, whether due to error or fraud. The Company is not required to have, nor were we engaged to perform, an audit of its internal control over financial reporting. As part of our audits we are required to obtain an understanding of internal control over financial reporting but not for the purpose of expressing an opinion on the effectiveness of the Company's internal control over financial reporting. Accordingly, we express no such opinion.

Our audits included performing procedures to assess the risks of material misstatement of the financial statements, whether due to error or fraud, and performing procedures that respond to those risks. Such procedures included examining, on a test basis, evidence regarding the amounts and disclosures in the financial statements. Our audits also included evaluating the accounting principles used and significant estimates made by management, as well as evaluating the overall presentation of the financial statements. We believe that our audits provide a reasonable basis for our opinion.

Critical Audit Matter

The critical audit matter communicated below is a matter arising from the current period audit of the financial statements that was communicated or required to be communicated to the audit committee and that: (1) relates to accounts or disclosures that are material to the financial statements and (2) involved our especially challenging, subjective or complex judgments. The communication of the critical audit matter does not alter in any way our opinion on the consolidated financial statements, taken as a whole, and we are not, by communicating the critical audit matter below, providing a separate opinion on the critical audit matter or on the accounts or disclosures to which they relate.

Valuation of Contingent Consideration and Approved Product

Description of the Matter

As described in Note R to the consolidated financial statements, on November 17, 2023, the Company completed its acquisition of Acer Therapeutics, Inc. in a transaction accounted for as a business combination. The total purchase consideration was approximately $73 million (including $8.5 million of contingent consideration) and the net assets acquired included a $68 million intangible asset related to the approved product (OLPRUVA).

Auditing the Company's valuations of the contingent consideration and OLPRUVA required auditor judgement due to the significant estimation utilized in the Company's determination of the fair values. The significant estimation was primarily due to the valuation models used by management to measure the fair values of the contingent consideration and the approved product as well as the subjectivity of the significant underlying assumptions. The Company utilized a monte carlo simulation method to estimate the fair value of the contingent consideration and the significant assumptions used in the model were the forecasted revenues, the discount rate and the success probabilities. The Company used a discounted cash flow model to measure the fair value of OLPRUVA and the significant assumptions used in the model included forecasted revenues and the discount rate.

How We Addressed the Matter in Our Audit

To test the fair value estimates of the contingent consideration and the approved product, we performed audit procedures which included, among others, evaluating the prospective financial information ("PFI") used in the valuation models, testing the completeness and accuracy of the underlying data and evaluating the Company's use of valuation methodologies. Our procedures to assess the PFI used in the valuation models, included, among others, evaluating the significant assumptions discussed above, by comparing them to industry and economic publications and trends, analyst reports and other objective sources. We involved our valuation specialists to assist in our evaluation of the reasonableness of the significant assumptions used in the fair value estimates as well as to independently calculate fair value estimates for the contingent consideration and OLPRUVA to compare to the Company's recorded amounts.

/s/ Ernst & Young LLP

We have served as the Company's auditor since 2022.

Orlando, Florida

April 1, 2024

ZEVRA THERAPEUTICS, INC.
CONSOLIDATED BALANCE SHEETS
(in thousands, except share and par value amounts)

	December 31,	
	2023	**2022 (As Restated)**
Assets		
Current assets:		
Cash and cash equivalents	$ 43,049	$ 65,466
Securities at fair value	24,688	16,900
Short-term investments - other	—	481
Accounts and other receivables	17,377	8,299
Prepaid expenses and other current assets	1,824	1,688
Total current assets	86,938	92,834
Inventories	9,841	671
Property and equipment, net	736	794
Operating lease right-of-use assets	790	988
Goodwill	4,701	—
Long-term investments - other	—	20,000
Intangible assets, net	69,227	—
Other long-term assets	94	53
Total assets	$ 172,327	$ 115,340
Liabilities and stockholders' equity		
Current liabilities:		
Accounts payable and accrued expenses	$ 28,403	$ 6,169
Line of credit payable	37,700	—
Current portion of operating lease liabilities	543	480
Current portion of discount and rebate liabilities	4,550	4,655
Other current liabilities	2,524	719
Total current liabilities	73,720	12,023
Line of credit payable	—	12,800
Secured promissory note	5,066	—
Derivative and warrant liability	16,100	10,202
Operating lease liabilities, less current portion	456	843
Discount and rebate liabilities, less current portion	7,663	4,327
Other long-term liabilities	7,458	25
Total liabilities	110,463	40,220
Commitments and contingencies (Note I)		
Stockholders' equity:		
Preferred stock:		
Undesignated preferred stock, $0.0001 par value, 10,000,000 shares authorized, no shares issued or outstanding as of December 31, 2023 or December 31, 2022	—	—
Common stock, $0.0001 par value, 250,000,000 shares authorized, 43,110,360 shares issued and 41,534,668 shares outstanding as of December 31, 2023; 35,450,257 shares issued and 34,540,304 shares outstanding as of December 31, 2022	4	3
Additional paid-in capital	472,664	436,269
Treasury stock, at cost	(10,983)	(7,536)
Accumulated deficit	(399,778)	(353,729)
Accumulated other comprehensive income	(43)	113
Total stockholders' equity	61,864	75,120
Total liabilities and stockholders' equity	$ 172,327	$ 115,340

See accompanying notes to consolidated financial statements

ZEVRA THERAPEUTICS, INC.
CONSOLIDATED STATEMENTS OF OPERATIONS
(in thousands, except share and per share amounts)

	Year Ended December 31,	
	2023	**2022 (As Restated)**
Revenue, net	$ 27,461	$ 10,161
Operating expenses:		
Cost of revenue	2,945	222
Research and development	39,806	19,803
Selling, general and administrative	34,314	15,038
Acquired in-process research and development	—	17,663
Total operating expenses	77,065	52,726
Loss from operations	(49,604)	(42,565)
Other (expense) income:		
Interest expense	(1,501)	(335)
Fair value adjustment related to derivative and warrant liability and CVR liability	(98)	15,159
Fair value adjustment related to investments	613	(577)
Interest and other income, net	4,541	1,513
Total other income	3,555	15,760
Loss before income taxes	(46,049)	(26,805)
Income tax (expense) benefit	—	33
Net loss	$ (46,049)	$ (26,772)
Basic and diluted net loss per share of common stock:		
Net loss	$ (1.30)	$ (0.78)
Weighted average number of shares of common stock outstanding:		
Basic and diluted	35,452,460	34,488,800

See accompanying notes to consolidated financial statements

ZEVRA THERAPEUTICS, INC.
CONSOLIDATED STATEMENTS OF COMPREHENSIVE INCOME (LOSS)
(in thousands)

	Year Ended December 31,	
	2023	**2022**
Net loss	$ (46,049)	$ (26,772)
Other comprehensive income:		
Foreign currency translation adjustment	(156)	113
Other comprehensive income (loss)	(156)	113
Comprehensive loss	$ (46,205)	$ (26,659)

See accompanying notes to consolidated financial statements

ZEVRA THERAPEUTICS, INC.
CONSOLIDATED STATEMENTS OF CHANGES IN STOCKHOLDERS' EQUITY
(in thousands)

	Common Stock	Additional Paid-in Capital	Treasury Stock	Accumulated Deficit	Other Comprehensive Income (Loss)	Total Stockholders' (Deficit) Equity
Balance as of January 1, 2022 (As Reported)	$ 4	$ 396,957	$ (2,814)	$ (267,029)	—	$ 127,118
Effect of restatement	—	34,470	—	(59,928)		(25,458)
Balance as of January 1, 2022 (As Restated)	$ 4	$ 431,427	$ (2,814)	$ (326,957)	$ —	$ 101,660
Net loss	—	—	—	(26,772)	—	(26,772)
Stock-based compensation expense	—	4,294	—	—	—	4,294
Shares repurchased as part of the Share Repurchase Program	(1)	—	(4,722)	—	—	(4,723)
Issuance of common stock as part of the Employee Stock Purchase Plan	—	324	—	—	—	324
Issuance of common stock in exchange for consulting services	—	224	—	—	—	224
Other comprehensive income	—	—	—	—	113	113
Balance as of December 31,2022 (As Restated)	$ 3	$ 436,269	$ (7,536)	$ (353,729)	$ 113	$ 75,120
Net loss	—	—	—	(46,049)	—	(46,049)
Stock-based compensation expense	—	5,954	—	—	—	5,954
Issuance of common stock in connection with the Merger	1	28,390	—	—	—	28,391
Issuance of common stock	—	6,106	—	—	—	6,106
Issuance of warrants	—	(4,500)	—	—	—	(4,500)
Shares repurchased as part of the Share Repurchase Program	—	—	(3,447)	—	—	(3,447)
Issuance of common stock as part of the Employee Stock Purchase Plan	—	218	—	—	—	218
Issuance of common stock in exchange for consulting services	—	227	—	—	—	227
Other comprehensive income (loss)	—	—	—	—	(156)	(156)
Balance as of December 31, 2023	$ 4	$ 472,664	$ (10,983)	$ (399,778)	$ (43)	$ 61,864

See accompanying notes to consolidated financial statements

ZEVRA THERAPEUTICS, INC.
CONSOLIDATED STATEMENTS OF CASH FLOWS
(in thousands)

	Year Ended December 31,	
	2023	**2022 (As Restated)**
Cash flows from operating activities:		
Net loss	$ (46,049)	$ (26,772)
Adjustments to reconcile net loss to net cash used in operating activities:		
Stock-based compensation expense	5,954	4,294
Depreciation and amortization expense	1,006	944
Fair value adjustment related to derivative and warrant liability and CVR liability	98	(15,159)
Fair value adjustment related to investments	(613)	577
Loss on sublease and disposal of property and equipment	157	9
Consulting fees paid in common stock	227	224
Acquired in-process research and development	—	17,663
Gain on foreign currency exchange rates	(198)	(91)
Change in assets and liabilities:		
Accounts and other receivables	(9,078)	(6,772)
Prepaid expenses and other current assets	152	(894)
Inventories	206	147
Operating lease right-of-use assets	326	277
Other long-term assets	(5)	386
Accounts payable and accrued expenses	11,130	3,131
Discount and rebate liabilities	3,231	3,782
Operating lease liabilities	(439)	(389)
Other liabilities	360	(74)
Net cash used in operating activities	(33,535)	(18,717)
Cash flows from investing activities:		
Acquisitions, net	(30,401)	(14,090)
Purchases of property and equipment	(296)	(93)
Purchases of investments	(45,814)	(23,861)
Maturities of investments	59,121	1,325
Net cash used in investing activities	(17,390)	(36,719)
Cash flows from financing activities:		
Proceeds from issuance of debt	42,431	12,800
Proceeds from insurance financing arrangements	1,256	1,273
Proceeds from Employee Stock Purchase Plan	218	324
Proceeds from stock issuance	6,106	—
Payments of principal on insurance financing arrangements	(564)	(1,306)
Repayment of debt	(17,531)	—
Payment to repurchase shares as part of the Share Repurchase Program	(3,447)	(4,723)
Repayment of principal on finance lease liabilities	(5)	(16)
Net cash provided by financing activities	28,464	8,352
Effect of exchange rate changes on cash and cash equivalents	44	204
Net decrease in cash and cash equivalents	(22,417)	(46,880)
Cash and cash equivalents, beginning of period	65,466	112,346
Cash and cash equivalents, end of period	$ 43,049	$ 65,466
Supplemental cash flow information:		
Cash paid for interest	$ 456	$ 321
Right-of-use assets obtained in exchange for lease liabilities	—	123
Supplemental disclosure of noncash investing activities:		
Issuance of common stock in connection with the Merger (Note R)	(28,390)	—
Supplemental disclosure of noncash financing activities:		
Issuance of secured promissory note for the Merger (Note R)	(5,066)	—
Issuance of contingent value rights for the Merger (Note R)	(8,562)	—

See accompanying notes to consolidated financial statements

ZEVRA THERAPEUTICS, INC.
NOTES TO CONSOLIDATED FINANCIAL STATEMENTS

A. Description of Business, Basis of Presentation, and Significant Transactions

Organization

Zevra Therapeutics, Inc. (the "Company") is a rare disease company combining science, data and patient needs to create transformational therapies for diseases with limited or no treatment options. The Company has a diverse portfolio of products and product candidates, which includes preclinical development programs, clinical stage pipeline and commercial stage assets. The Company's pipeline includes arimoclomol, an orally-delivered, first-in-class investigational product candidate being developed for Niemann-Pick disease type C ("NPC"), which has been granted orphan drug designation, Fast-track designation, Breakthrough Therapy designation and rare pediatric disease designation for the treatment of NPC by the U.S. Food and Drug Administration ("FDA") and orphan medical product designation for the treatment of NPC by the European Medicines Agency ("EMA"). The arimoclomol NDA for NPC was resubmitted to the FDA on December 21, 2023, and has been assigned a PDUFA date of September 21, 2024. KP1077 is the Company's lead clinical development product candidate which is being developed as a treatment for idiopathic hypersomnia ("IH"), a rare neurological sleep disorder, and narcolepsy. KP1077 is comprised solely of serdexmethylphenidate ("SDX"), the Company's proprietary prodrug of d-methylphenidate ("d-MPH"). The FDA has granted KP1077 orphan drug designation for the treatment of IH. OLPRUVA® (sodium phenylbutyrate) for oral suspension is approved by the FDA for the treatment of urea cycle disorders ("UCDs"). The Company also has a pipeline of investigational product candidates, including celiprolol for the treatment of vascular Ehlers-Danlos syndrome in patients with a confirmed type III collagen mutation.

The Company changed its name from KemPharm, Inc. to Zevra Therapeutics, Inc. effective as of February 21, 2023. On March 1, 2023, following its name change, the Company's common stock began trading on the Nasdaq Global Select Market under the ticker symbol "ZVRA".

Going Concern

The accompanying consolidated financial statements have been prepared on a going concern basis, which contemplates the realization of assets and the satisfaction of liabilities in the normal course of business. During the year ended December 31, 2023, the Company incurred a net loss of $46.0 million and, as of December 31, 2023, has an accumulated deficit of $399.8 million as well as cash and investments on hand of $67.7 million. The Company has sustained operating losses for the majority of its corporate history and expects to continue to incur operating losses and negative operating cash flows until revenues reach a level sufficient to support ongoing operations. The Company's liquidity needs will be largely determined by the success of operations through the progression of its product candidates in the future. The Company also may consider other sources to fund operations including: (1) out-licensing rights to certain of its technologies and product candidates, pursuant to which the Company would receive cash royalties and milestones; (2) raising additional capital through equity or debt financings or from other sources; (3) obtaining product candidate regulatory approvals, which would generate revenue, milestones and cash flow; (4) reducing spending on one or more research and development programs, including by discontinuing development; and/or (5) restructuring operations to change its overhead structure.

The Company's ability to continue operating as a going concern is contingent upon its ability to secure sufficient financing and/or reduce spending to maintain operations. Unless the Company is able to restructure the amounts outstanding on its margin loan facility, we may be required to repay the loan and thereby deplete the cash available to fund our operations. If this occurs, our forecasts reflect a shortfall in cash available for operations as early as mid-2024. While the Company expects to obtain the necessary financing that is needed, there is no assurance that the Company will be successful in obtaining the necessary funding for future operations. These factors raise substantial doubt as to the Company's ability to continue as a going concern for at least one year from the date these financial statements are being issued. The financial statements do not include any adjustments that might result from the outcome of these uncertainties.

Basis of Presentation

The Company prepared the consolidated financial statements in accordance with accounting principles generally accepted in the United States of America ("US GAAP") and the rules and regulations of the Securities and Exchange Commission ("SEC") and, in the Company's opinion, reflect all adjustments, including normal recurring items that are necessary. All significant intercompany accounts and transactions have been eliminated in consolidation.

Merger

On August 30, 2023, the Company and Aspen Z Merger Sub, Inc., a wholly-owned subsidiary of Zevra ("Merger Sub"), entered into an Agreement and Plan of Merger (the "Merger Agreement") with Acer Therapeutics Inc. ("Acer"), a pharmaceutical company focused on development and commercialization of therapies for rare and life-threatening diseases. On November 17, 2023 (the "Closing Date"), the Company completed the acquisition of Acer. Pursuant to the Merger Agreement, on the Closing Date, Merger Sub was merged with and into Acer (the "Merger"), with Acer continuing as the surviving entity and as a wholly-owned subsidiary of Zevra. In connection therewith, Zevra has also purchased Acer's secured debt from Nantahala Capital Management, LLC ("NCM"), certain of its affiliates and certain other parties (collectively with NCM, "Nantahala") through a series of transactions and Zevra agreed to provide Acer with a bridge loan facility for up to $18.0 million ("Bridge Loan"), subject to certain terms and conditions. The Merger has expanded Zevra's rare disease portfolio, as well as increased and diversified its revenues with the addition of a U.S. commercial asset, OLPRUVA, indicated for the treatment of UCDs. See Note R for further discussion related to the Merger.

Arimoclomol Acquisition

On May 15, 2022, the Company and Zevra Denmark A/S ("Zevra DK"), a newly formed Danish company and wholly-owned subsidiary of the Company entered into an asset purchase agreement (the "Arimoclomol Purchase Agreement") with Orphazyme A/S in restructuring, a Danish public limited liability company ("Orphazyme"). The Arimoclomol Purchase Agreement closed on May 31, 2022. Under the terms of the Arimoclomol Purchase Agreement, Zevra DK purchased all of the assets and operations of Orphazyme related to arimoclomol and settled all of Orphazyme's actual outstanding liabilities to its creditors with a cash payment of $12.8 million. In addition, Zevra DK agreed to assume an estimated reserve liability of $5.2 million related to revenue generated from Orphazyme's Expanded Access Program in France.

The Company accounted for the arimoclomol acquisition as an asset acquisition as the majority of the value of the assets acquired related to the arimoclomol acquired in-process research and development ("IPR&D") asset. The intangible asset associated with IPR&D relates to arimoclomol. The estimated fair value of $17.7 million was determined using the excess earnings valuation method, a variation of the income valuation approach. The excess earnings valuation method estimates the value of an intangible asset equal to the present value of the incremental after-tax cash flows attributable to that intangible asset over its remaining economic life. Some of the more significant assumptions utilized in our asset valuations included projected revenues, probability of commercial success, and the discount rate. The fair value using the excess earnings valuation method was determined using an estimated weighted average cost of capital of 42%, which reflects the risks inherent in future cash flow projections and represents a rate of return that a market participant would expect for this asset. This fair value measurement was based on significant inputs not observable in the market and thus represent Level 3 fair value measurement.

In accordance with Accounting Standards Codification (ASC) Subtopic 730-10-25, *Accounting for Research and Development Costs*, the up-front payments to acquire a new drug compound, as well as future milestone payments when paid or payable, are immediately expensed as acquired IPR&D in transactions other than a business combination provided that the drug has not achieved regulatory approval for marketing and, absent obtaining such approval, has no alternative future use. Therefore, the portion of the purchase price that was allocated to the IPR&D assets acquired was immediately expensed. Other assets acquired and liabilities assumed, were recorded at fair value. The company also recorded a $0.8 million income tax benefit for the year-ended December 31, 2022, related to research and development credits that are expected to be realized from the local jurisdiction in Denmark.

The following represents the consideration paid and purchase price allocation for the acquisition of arimoclomol (in thousands):

Cash	$	12,800
Assumed reserve liability		5,200
Total consideration	$	18,000
Total consideration		18,000
Direct transaction costs associated with the acquisition (1)		1,290
Total purchase price to be allocated	**$**	**19,290**
Property and equipment, inventory and assembled workforce acquired		1,627
IPR&D (2)		17,663
Total allocated purchase price	**$**	**19,290**

(1) As a result of the asset acquisition accounting, the transaction costs associated with the acquisition should be included in the costs of the assets acquired and allocated amongst qualifying assets using the relative fair value basis. The transaction costs primarily included financial advisor fees and legal expenses.
(2) The primary asset acquired, the IPR&D asset, was expensed and the allocated transaction related costs were included with and expensed with this asset.

Amendment to Registration Statement on Form S-3

On January 25, 2022, the Company filed an amendment to the registration statement on Form S-1 (File No. 333-250945) on Form S-3 covering the issuance of the shares of our common stock issuable upon the exercise of the warrants issued in the Public Offering and remaining unexercised as of the date of the amendment, which was declared effective on February 1, 2022.

In connection with the Merger, Zevra and Nantahala (as defined in Note R) concurrently entered into a registration rights agreement, pursuant to which Zevra agreed to file a resale registration statement with respect to the resale of the Zevra common stock issuable to Nantahala. On February 5, 2024, Zevra filed a registration statement on Form S-3 (File No. 333-276856) registering an aggregate of 2,269,721 shares of Zevra's common stock.

Entry into 2021 ATM Agreement

On July 2, 2021, the Company entered into an equity distribution agreement (the "2021 ATM Agreement") with JMP Securities LLC ("JMP") and RBC Capital Markets, LLC ("RBCCM") under which the Company may offer and sell, from time to time at its sole discretion, shares of its common stock having an aggregate offering price of up to $75.0 million through JMP and RBCCM as its sales agents. The issuance and sale, if any, of common stock by the Company under the 2021 ATM Agreement will be made pursuant to a registration statement on Form S-3. JMP and RBCCM may sell the common stock by any method permitted by law deemed to be an "at the market offering" as defined in Rule 415 of the Securities Act of 1933, as amended. JMP and RBCCM will use commercially reasonable efforts to sell the common stock from time to time, based upon instructions from the Company (including any price, time or size limits or other customary parameters or conditions the Company may impose). The Company will pay JMP and RBCCM a commission equal to 3.0% in the aggregate of the gross sales proceeds of any common stock sold through JMP and RBCCM under the 2021 ATM Agreement. The Company filed a registration statement on Form S-3 covering the sale of the shares of its common stock up to $350.0 million, $75.0 million of which was allocated to the sales of the shares of common stock issuable under the 2021 ATM Agreement, which was declared effective on July 12, 2021. As of December 31, 2023 and 2022, no shares have been issued or sold under the 2021 ATM Agreement.

Share Repurchase Program

On December 20, 2021, the Company initiated a share repurchase program (the "Share Repurchase Program") pursuant to which the Company may repurchase up to $50 million of shares of its common stock through December 31, 2023. On December 31, 2023, the Share Repurchase Program ended, and the Company had repurchased 1,575,692 shares of its common stock for approximately $11.0 million.

Reclassifications

Certain reclassifications were made to the 2022 consolidated financial statements to conform to the classifications used in 2023. These reclassifications had no impact on the consolidated net loss changes in stockholder's equity, or cash flows previously reported.

B. Summary of Significant Accounting Policies

Use of Estimates

The preparation of these consolidated financial statements in conformity with U.S. GAAP requires the Company to make estimates and assumptions that affect the amounts reported in the consolidated financial statements and accompanying notes. Actual results could differ from those estimates.

On an ongoing basis, the Company evaluates its estimates and assumptions, including those related to revenue recognition, the useful lives of property and equipment, the recoverability of long-lived assets, the incremental borrowing rate for leases, and assumptions used for purposes of determining stock-based compensation, income taxes, the fair value of long-term investments, the fair value of the derivative and warrant liability and discount and rebate liabilities, among others. The Company bases its estimates on historical experience and on various other assumptions that it believes to be reasonable, the results of which form the basis for making judgments about the carrying value of assets and liabilities.

Concentration of Credit Risk

Financial instruments that potentially expose the Company to concentrations of credit risk consist principally of cash on deposit and investments with multiple financial institutions, the balances of which frequently exceed insured limits, and accounts receivable, which are concentrated amongst a limited number of customers.

Cash and Cash Equivalents

The Company considers any highly liquid investments with an original maturity of three months or less to be cash equivalents.

Investments

The Company maintains investment securities that are classified as available-for-sale securities for which the Company has elected the fair value option under ASC 825, *Financial Instruments*. As such, these securities are carried at fair value with unrealized gains and losses included in fair value adjustment related to investments on the consolidated statements of operations. The securities primarily consist of U.S. Treasury securities and U.S. government-sponsored agency securities and are included in securities at fair value in the consolidated balance sheets. As of December 31, 2023, and 2022, the Company held securities with an aggregate fair value of $24.7 million and $16.9 million, respectively, that contained aggregate unrealized gains of approximately $0.6 million and unrealized losses of $0.6 million, respectively. Applying fair value accounting to these debt securities more accurately represents the Company's investment strategy due to the fact that excess cash is currently being invested for the purpose of funding future operation **s.** In addition, the Company held certificates of deposit totaling $20.5 million as of December 31, 2022, which are included in investments - other in the consolidated balance sheet as of December 31, 2022. These certificates of deposit matured in May 2023. Interest income is recognized as earned using an effective yield method giving effect to the amortization of premium and accretion of discount and is based on the economic life of the securities Interest income is included in Interest and other income, net in the consolidated statements of operations.

Variable Interest Entities

The primary beneficiary of a variable interest entity ("VIE") is required to consolidate the assets and liabilities of the VIE. When the Company obtains a variable interest in another entity, it assesses at the inception of the relationship and upon occurrence of certain significant events whether the entity is a VIE, and if so, whether the Company is the primary beneficiary of the VIE based on its power to direct the activities of the VIE that most significantly impact the VIE's economic performance and the Company's obligation to absorb losses or the rights to receive benefits from the VIE that could potentially be significant to the VIE.

To assess whether the Company has the power to direct the activities of the VIE that most significantly impact the VIE's economic performance, the Company considers all the facts and circumstances, including the Company's role in establishing the VIE and the Company's ongoing rights and responsibilities. The assessment includes identifying the activities that most significantly impact the VIE's economic performance and identifying which party, if any, has the power to direct those activities. In general, the parties that make the most significant decisions affecting the VIE (management and representation on the Board of Directors) are deemed to have the power to direct the activities of a VIE.

To assess whether the Company has the obligation to absorb losses of the VIE or the rights to receive benefits from the VIE that could potentially be significant to the VIE, the Company considers all of its economic interests that are deemed to be variable interests in the VIE.

This assessment requires judgement in determining whether these interests, in the aggregate, are considered potentially significant to the VIE. As of December 31, 2023, the Company identified Acer to be the Company's sole interest in a VIE (Note R). As Zevra is the final decision maker for all of Acer's research, development, and commercialization of drug candidates that it is producing, the Company directs the activities of Acer that most significantly impact its performance. Therefore, the Company is the primary beneficiary of this VIE for accounting purposes. The Company did not identify any interests in VIE's as of December 31, 2022.

Goodwill and Intangible Assets

Goodwill represents the excess of the consideration transferred over the fair value of the net assets acquired when accounted for using the acquisition method of accounting for business combinations. Goodwill is not amortized but is evaluated for impairment within the Company's single reporting unit on an annual basis, during the fourth quarter, or more frequently if an event occurs or circumstances change that would more-likely-than-not reduce the fair value of the Company's reporting unit below its carrying amount. The Company establishes its reporting units based on the organizational structure and has determined it has one reporting unit. In performing its analysis, in accordance with ASC 35, the Company has the option to first assess qualitatively whether it is more likely than not that the fair value of a reporting unit is less than its carrying amount, including goodwill. In performing qualitative assessments, the Company considers, among other factors, macroeconomic conditions, the Company's overall financial performance (including, but not limited to, comparisons to prior periods, current period internal expectations, and comparable peer companies), broader industry and market considerations, and the trading price performance of the Company's common stock.

The Company's goodwill balance was $4.7 million as of December 31, 2023. There was no goodwill recorded as of December 31, 2022. As of December 31, 2022, the Company completed its annual qualitative assessment under ASC 350 to determine whether the existence of events or circumstances indicated that it was more likely than not that the fair value of its reporting unit was less than its respective carrying value. The Company concluded that based on the relevant events and circumstances, it was more likely than not that the reporting unit's fair value exceeded its related carrying value and therefore no quantitative assessment was required. No goodwill impairment charges were recorded for the year ended December 31, 2023.

Acquired in-process research and development ("IPR&D") that the Company acquires in conjunction with the acquisition of a business represents the fair value assigned to incomplete research projects which, at the time of acquisition, have not reached technological feasibility. The amounts are capitalized and are accounted for as indefinite-lived intangible assets, subject to impairment testing until completion or abandonment of the projects. Upon successful completion of each IPR&D project, the Company will make a determination as to the then-useful life of the intangible asset, generally determined by the period in which the substantial majority of the cash flows are expected to be generated and begin amortization. The Company evaluates IPR&D for impairment on an annual basis, during the fourth quarter, or more frequently if impairment indicators exist. The Company's IPR&D balance was $2.0 million as of December 31, 2023. There was no IPR&D asset included in the consolidated balance sheet as of December 31, 2022, as the IPR&D asset acquired in May 2022 under the Arimoclomol Purchase Agreement was immediately expensed (Note A). No IPR&D impairment charges were recognized for the years ended December 31, 2023, or 2022.

As of December 31, 2023, the Company had a $67.2 million definite-lived intangible asset, net related to the acquisition of OLPRUVA as a result of the Merger. This is amortized on a straight-line basis over its estimated economic life of eleven years and is reviewed periodically for impairment. Amortization expense is recorded as a component of cost of revenue in the consolidated statements of operations and was $0.8 million for the year-ended December 31, 2023.

For intangible assets subject to amortization, estimated amortization expense for the five fiscal years subsequent to December 31, 2023, is as follows (in thousands):

2024	$	6,182
2025	$	6,182
2026	$	6,182
2027	$	6,182
2028	$	6,182

Property and Equipment

The Company records property and equipment at cost less accumulated depreciation and amortization. Costs of renewals and improvements that extend the useful lives of the assets are capitalized. Maintenance and repairs are expensed as incurred. Depreciation is determined on a straight-line basis over the estimated useful lives of the assets, which generally range from three to ten years. Leasehold improvements are amortized over the shorter of the useful life of the asset or the term of the related lease. Upon retirement or disposition of assets, the costs and related accumulated depreciation and amortization are removed from the accounts with the resulting gains or losses, if any, reflected in the consolidated statements of operations.

Impairment of Long-Lived Assets

Long-lived assets to be held and used are reviewed for impairment whenever events or changes in circumstances indicate that the carrying amounts of the assets may not be recoverable. When such events occur, the Company compares the carrying amounts of the assets to their undiscounted expected future cash flows. If the undiscounted cash flows are insufficient to recover the carrying values, an impairment loss is recorded for the difference between the carrying values and fair values of the asset. No such impairment occurred for the years ended December 31, 2023, or 2022.

Revenue Recognition

The Company recognizes revenue in accordance with ASC 606, *Revenue from Contracts with Customers* ("ASC 606") and, as a result, follows the five-step model when recognizing revenue: 1) identifying a contract; 2) identifying the performance obligations; 3) determining the transaction price; 4) allocating the price to the performance obligations; and 5) recognizing revenue when the performance obligations have been fulfilled.

Arimoclomol Expanded Access Program

Net revenue includes revenue from the sale of arimoclomol for the treatment of NPC under the remunerated expanded access compassionate use program in France (“French nATU”). An expanded access compassionate use program is a program giving specific patients access to a drug, which is not yet approved for commercial sale. Only drugs targeting serious or rare indications and for which there is currently no appropriate treatment are considered for expanded access compassionate use programs. Further, to be considered for the expanded access compassionate use program, the drug must have proven efficacy and safety and must either be undergoing price negotiations or seeking marketing approval.

In accordance with ASC 606, the Company recognizes revenue when fulfilling its performance obligation under the Arimoclomol Expanded Access Program ("Arimoclomol EAP") by transferring control of promised goods or services to its customer, in an amount that reflects the consideration that the Company expects to receive in exchange for those goods or services. In determining when the customer obtains control of the product, the Company considers certain indicators, including whether the Company has a present right to payment from the customer, whether title and/or significant risks and rewards of ownership have transferred to the customer and whether customer acceptance has been received. Revenue is recognized net of sales deductions, including discounts, rebates, applicable distributor fees, and revenue-based taxes.

The French Health Authorities and the manufacturer have agreed to a price for sales during the French nATU, but the final transaction price depends on the terms and conditions in the contracts with the French Health Authorities, following market approval. Any excess in the price charged by the manufacturer compared to the price agreed with the health authorities once the drug product is approved in France must be repaid. The repayment is considered in the clawback liability (rebate). An estimate of net revenue and clawback liability are recognized using the ‘expected value’ method. Accounting for net revenue and clawback liability requires determination of the most appropriate method for the expected final transaction price. This estimate also requires assumptions with respect to inputs into the method, including current pricing of comparable marketed products within the rare disease area in France. Management has considered the expected final sales price as well as the price of similar drug products. The Company is operating within a rare disease therapeutic area where there is unmet treatment need and hence a limited number of comparable commercialized drug products. The limited available relevant market information for directly comparable commercialized drugs within rare disease increases the uncertainty in management's estimate.

For the years ended December 31, 2023, and 2022, the Company recognized revenue related to the Arimoclomol EAP in France of $8.7 million and $4.8 million, respectively, which is net of newly accrued clawback liability for each respective year of $5.1 million and $3.8 million, respectively, and other gross to net adjustments. As part of the Arimoclomol Purchase Agreement the Company assumed an estimated reserve liability of $5.2 million related to revenue generated from the Arimoclomol EAP in France. The total estimate reserve liability as of December 31, 2023, and December 31, 2022, including the additional clawback liability for the year ended December 31, 2023, and December 31, 2022, was $12.2 million and $9.0 million, respectively. As of December 31, 2023, and 2022, this estimated reserve liability is recorded as discount and rebate liabilities in the consolidated balance sheets and is separated into current and long-term based upon the timing of the expected payment to the French regulators.

Licensing Agreements

The Company enters into licensing agreements with licensees that fall under the scope of ASC 606.

The terms of the Company's licensing agreements typically include one or more of the following: (i) upfront fees; (ii) milestone payments related to the achievement of development, regulatory, or commercial goals; and (iii) royalties on net sales of licensed products. Each of these payments may result in licensing revenues.

As part of the accounting for these agreements, the Company must develop estimates and assumptions that require judgment to determine the underlying stand-alone selling price for each performance obligation which determines how the transaction price is allocated among the performance obligations. Generally, the estimation of the stand-alone selling price may include such estimates as, independent evidence of market price, forecasted revenues or costs, development timelines, discount rates, and probability of regulatory success. The Company evaluates each performance obligation to determine if they can be satisfied at a point in time or over time, and it measures the services delivered to the licensee which are periodically reviewed based on the progress of the related program. The effect of any change made to an estimated input component and, therefore revenue or expense recognized, would be recorded as a change in estimate. In addition, variable consideration (e.g., milestone payments) must be evaluated to determine if it is constrained and, therefore, excluded from the transaction price.

Up front Fees: If a license to the Company's intellectual property is determined to be distinct from the other performance obligations identified in the arrangement, the Company recognizes revenues from the transaction price allocated to the license when the license is transferred to the licensee and the licensee is able to use and benefit from the license. For licenses that are bundled with other promises, the Company utilizes judgment to assess the nature of the combined performance obligation to determine whether the combined performance obligation is satisfied over time or at a point in time.

Milestone Payments: At the inception of each arrangement that includes milestone payments (variable consideration), the Company evaluates whether the milestones are considered probable of being reached and estimates the amount to be included in the transaction price using the most likely amount method. If it is probable that a significant revenue reversal would not occur, the associated milestone value is included in the transaction price. Milestone payments that are not within the Company's or the licensee's control, such as non-operational developmental and regulatory approvals, are generally not considered probable of being achieved until those approvals are received. At the end of each reporting period, the Company re-evaluates the probability of achievement of milestones that are within its or the licensee's control, such as operational developmental milestones and any related constraint, and if necessary, adjusts its estimate of the overall transaction price. Any such adjustments are recorded on a cumulative catch-up basis, which would affect collaboration revenues and earnings in the period of adjustment. Revisions to the Company's estimate of the transaction price may also result in negative licensing revenues and earnings in the period of adjustment.

AZSTARYS License Agreement

The Company entered into a Collaboration and License Agreement (the "AZSTARYS License Agreement") with Commave Therapeutics SA ("Commave"), an affiliate of GPC. Under the AZSTARYS License Agreement, as amended, the Company granted to Commave an exclusive, worldwide license to develop, manufacture and commercialize the Company's product candidates containing SDX and d-methylphenidate ("d-MPH"), including AZSTARYS, or any other product candidate developed by the Company containing SDX and developed to treat ADHD or any other CNS disorder. Corium was tasked by Commave, to lead all commercialization activities for AZSTARYS under the AZSTARYS License Agreement. Pursuant to the AZSTARYS License Agreement, Corium agreed to pay milestone payments up to an aggregate of $590.0 million upon the occurrence of specified regulatory milestones related to AZSTARYS, additional fixed payments upon the achievement of specified U.S. sales milestones, and quarterly, tiered royalty payments based on a range of percentages of net sales (as defined in the AZSTARYS License Agreement). Commave is obligated to make such royalty payments on a product-by-product basis until expiration of the royalty term for the applicable product.

The AZSTARYS License Agreement is within the scope of ASC 606, as the transaction represents a contract with a customer where the participants function in a customer / vendor relationship and are not exposed equally to the risks and rewards of the activities contemplated under the AZSTARYS License Agreement. The Company concluded that these regulatory milestones, sales milestones and royalty payments each contain a significant uncertainty associated with a future event. As such, these milestone and royalty payments are constrained at contract inception and are not included in the transaction price as the Company could not conclude that it is probable a significant reversal in the amount of cumulative revenue recognized will not occur surrounding these milestone payments. At the end of each reporting period, the Company updates its assessment of whether the milestone and royalty payments are constrained by considering both the likelihood and magnitude of the potential revenue reversal. For the year ended December 31, 2023, the Company recognized revenue under the AZSTARYS License Agreement of $18 .5 million, which includes $15.0 million in net sales milestone payments earned during the year then ended. For the year ended December 31, 2022, the Company recognized $0.9 million under the AZSTARYS License Agreement. There was no deferred revenue related to this agreement as of December 31, 2023, and 2022.

In accordance with the terms of the Company's Termination Agreement with Aquestive Therapeutics dated March 20, 2012, Aquestive Therapeutics ("Aquestive") has the right to receive an amount equal to 10% of any royalty or milestone payments made to the Company related to AZSTARYS, KP879 or KP1077 under the AZSTARYS License Agreement. The Company recorded $1.8 million and $0.1 million in royalty payments owed to Aquestive as of December 31, 2023, and 2022, respectively, which is included in cost of revenue in the consolidated statements of operations.

Relief License Agreement

As a condition to entering into the Merger Agreement, Acer and Relief Therapeutics, Inc. ("Relief") entered into an exclusive license agreement on August 30, 2023 (the "Relief License Agreement"). Pursuant to the Relief License Agreement, Relief will hold exclusive development and commercialization rights for OLPRUVA in the European Union, Liechtenstein, San Marino, Vatican City, Norway, Iceland, Principality of Monaco, Andorra, Gibraltar, Switzerland, United Kingdom, Albania, Bosnia, Kosovo, Montenegro, Serbia and North Macedonia ("Geographical Europe"). The Company will have the right to receive a royalty of up to 10.0% of the net sales of OLPRUVA in Geographical Europe. For the year ended December 31, 2023, the Company did not recognize any revenue under the Relief License Agreement. There was no deferred revenue related to this agreement as of December 31, 2023. For further discussion of the Relief License Agreement, see Note R.

Product Revenue, net

On December 27, 2022, the FDA approved OLPRUVA (sodium phenylbutyrate), a prescription medicine used along with certain therapy, including changes in diet, for the long-term management of adults and children with UCDs weighing 44 pounds (20 kg) or greater and with a body surface area of 1.2m2 or greater, On November 17, 2023, the Company acquired OLPRUVA in connection with the Merger (Note R). To commercialize OLPRUVA for oral suspension in the U.S. the Company is building marketing, sales, medical affairs, distribution, managerial and other non-technical capabilities or making arrangements with third parties to perform these services. The Company's current distributor for sales of OLPRUVA is a single specialty pharmacy provider. However, the Company intends to establish additional distributors such as other retail pharmacies and certain medical centers or hospitals. In addition to distribution agreements, the Company enters into arrangements with health care providers and payors that provide for government mandated and/or privately negotiated rebates with respect to the purchase of it products. For the year ended December 31, 2023, the Company recognized negligible revenue related to sales of OLPRUVA.

Consulting Arrangements

The Company enters into consulting arrangements with third parties that fall under the scope of ASC 606. These arrangements may require the Company to deliver various rights, services, including research and development services, regulatory services and/or commercialization support services. The underlying terms of these arrangements generally provide for consideration to the Company in the form of consulting fees and reimbursements of out-of-pocket third-party research and development, regulatory and commercial costs.

Corium Consulting Agreement

In July 2020, the Company entered into a consultation services arrangement (the "Corium Consulting Agreement") with Corium, Inc. ("Corium") under which Corium engaged the Company to guide the product development and regulatory activities for certain current and potential future products in Corium's portfolio, as well as continue supporting preparation for the potential commercial launch of AZSTARYS (together, "Corium Consulting Services"). Corium is a portfolio company of GPC and was tasked by Commave to lead all commercialization activities for AZSTARYS under the AZSTARYS License Agreement, as discussed above.

Under the Corium Consulting Agreement, the Company received payments from Corium of $15.6 million, $13.6 million of which was paid in quarterly installments through March 31, 2022. The remaining $2.0 million was received in the first quarter of 2022 upon the approval by the FDA of Corium's product known as ADLARITY. The Corium Consulting Agreement is within the scope of ASC 606, as the transaction represents a contract with a customer where the participants function in a customer/vendor relationship and are not exposed equally to the risks and rewards of the activities contemplated under the Corium Consulting Agreement. The Company identified the performance of consulting services, which includes the reimbursement to the Company of third-party pass-through costs, as its only performance obligation at inception. The Company further determined that the transaction price, at inception, under the agreement was $13.6 million which is the fair value of the consulting services, including the reimbursement of third-party pass-through costs. The Company concluded that the regulatory milestone contains a significant uncertainty associated with a future event. As such, this milestone is constrained at contract inception and is not included in the transaction price as the Company could not conclude that it is probable a significant reversal in the amount of cumulative revenue recognized will not occur surrounding these milestone payments.

The Company determined that the performance of consulting services, including reimbursement of third-party pass-through costs, is a performance obligation that is satisfied over time as the services are performed and the reimbursable costs are paid. As such, the revenue related to the performance obligation will be recognized as the consulting services are performed and the services associated with the reimbursable third-party pass-through costs are incurred and paid by the Company, in accordance with the practical expedient allowed under ASC 606 regarding an entity's right to consideration from a customer in an amount that corresponds directly to the value to the customer of the entity's performance completed to date. As of December 31, 2022, the Company has recognized approximately all of the consulting services and third-party pass-through costs under the Corium Consulting Agreement.

For the years ended December 31, 2023, and 2022 the Company recognized revenue under the Corium Consulting Agreement of $0.2 million and $4.7 million, respectively. As of December 31, 2023, and 2022, the Company had no deferred revenue related to this agreement. The Corium Consulting Agreement expired on March 31, 2023.

Cost of Revenue

The components of cost of revenue are royalties and expenses directly attributable to revenue. To date, the Company has generated revenue from the AZSTARYS License Agreement, sales of arimoclomol under the Arimoclomol EAP, reimbursement of out-of-pocket third-party costs and the performance of consulting services. In connection with the AZSTARYS License Agreement, the Company pays Aquestive a royalty equal to 10% of all regulatory milestone and royalty payments. In addition, the Company capitalized incremental costs directly attributable to the AZSTARYS License Agreement. These costs are amortized to royalties and contract costs as revenue is recognized.

Accounts and Other Receivables

Accounts and other receivables consist of receivables under the AZSTARYS License Agreement and Arimoclomol EAP, as well as receivables related to consulting arrangements, income tax receivables and other receivables due to the Company. Receivables under the AZSTARYS License Agreement are recorded for amounts due to the Company related to reimbursable third-party costs as well as milestones and royalties on product sales. Receivables under the Arimoclomol EAP are recorded for product sales under the French nATU. The Company provides reserves against receivables for estimated losses that may result from a customer's inability to pay. Receivables are evaluated to determine if any reserve or allowance should be recorded based on consideration of the current economic environment, expectations of future economic conditions, specific circumstances and the Company's own historical collection experience. Amounts determined to be uncollectible are charged or written-off against the reserve.

Inventory

As discussed in Note A, a portion of the purchase price for Arimoclomol was allocated to inventory acquired representing the value of inventory associated with forecasted EAP revenues, which is recorded at its net realizable value. The Company determines the cost of its other inventories, which includes amounts related to materials and manufacturing overhead, on a first-in, first-out basis.

The Company may scale-up and make commercial quantities of its product candidates prior to the date it anticipates that such product will receive final regulatory approval. The scale-up and commercial production of pre-launch inventory involves the risk that such products may not be approved for marketing on a timely basis, or ever. This risk notwithstanding, the Company may scale-up and build pre-launch inventory of product that have not received final regulatory approval when the Company believes such action is appropriate in relation to the commercial value of the product launch opportunity. Inventory manufactured prior to regulatory approval is recorded as research and development expense until regulatory approval for the product is obtained. Inventory used in clinical trials is also expensed as research and development expense, when selected for such use. Inventory that can be used in either the production of clinical or commercial products is expensed as research and development costs when identified for use in a clinical manufacturing campaign. The cost of finished goods inventory that is shipped to a customer to support the Company's patient assistance programs is expensed when those shipments take place. As of December 31, 2023 and 2022, the Company did not have pre-launch inventory that qualified for capitalization.

The Company performs an assessment of the recoverability of capitalized inventory during each reporting period and writes down any excess and obsolete inventory to its net realizable value in the period in which the impairment is first identified. Such impairment charges, should they occur, are recorded as a component of cost of product sales in the statements of operations and comprehensive loss. The determination of whether inventory costs will be realizable requires the use of estimates by management. If actual market conditions are less favorable than projected by management, additional write-downs of inventory may be required. Additionally, the Company's product is subject to strict quality control and monitoring that it performs throughout the manufacturing process. In the event that certain batches or units of product do not meet quality specifications, the Company will record a charge to cost of product sales, to write-down any unmarketable inventory to its estimated net realizable value. The components of inventory are summarized as follows:

	December 31, 2023	December 31, 2022
Raw Materials	$ 2,938	$ —
Work in progress	1,884	—
Finished goods	5,019	671
Total inventory	$ 9,841	$ 671

Research and Development

Major components of research and development costs include cash compensation, stock-based compensation, depreciation and amortization expense on research and development property and equipment, costs of preclinical studies, clinical trials and related clinical manufacturing, costs of drug development, costs of materials and supplies, facilities cost, overhead costs, regulatory and compliance costs, and fees paid to consultants and other entities that conduct certain research and development activities on the Company's behalf. Costs incurred in research and development are expensed as incurred.

The Company records nonrefundable advance payments it makes for future research and development activities as prepaid expenses. Prepaid expenses are recognized as expense in the statements of operations as the Company receives the related goods or services.

The Company enters into contractual agreements with third-party vendors who provide research and development, manufacturing, and other services in the ordinary course of business. Some of these contracts are subject to milestone-based invoicing and services are completed over an extended period of time. The Company records liabilities under these contractual commitments when an obligation has been incurred. This accrual process involves reviewing open contracts and purchase orders, communicating with the applicable personnel to identify services that have been performed and estimating the level of service performed and the associated cost when the Company has not yet been invoiced or otherwise notified of actual cost. The majority of the service providers invoice the Company monthly in arrears for services performed. The Company makes estimates of the accrued expenses as of each balance sheet date based on the facts and circumstances known. The Company periodically confirms the accuracy of the estimates with the service providers and make adjustments, if necessary.

Patent Costs

Patent costs, including related legal costs, are expensed as incurred and recorded within general and administrative expenses on the statements of operations.

Income Taxes

The Company recognizes deferred tax assets and liabilities for the expected future tax consequences of temporary differences between the financial reporting and tax basis of assets and liabilities, as well as for operating loss and tax credit carryforwards. Deferred tax assets and liabilities are measured using the tax rates that are expected to apply to taxable income for the years in which those tax assets and liabilities are expected to be realized or settled. Valuation allowances are recorded to reduce deferred tax assets to the amount the Company believes is more likely than not to be realized.

Uncertain tax positions are recognized only when the Company believes it is more likely than not that the tax position will be upheld on examination by the taxing authorities based on the merits of the position. The Company recognizes interest and penalties, if any, related to unrecognized income tax uncertainties in income tax expense. The Company did not have any accrued interest or penalties associated with uncertain tax positions as of December 31, 2023, and 2022.

The Company files income tax returns in the United States for federal and various state jurisdictions. With few exceptions, the Company is no longer subject to U.S. federal and state and local income tax examinations for years prior to 2015, although carryforward attributes that were generated prior to 2017 may still be adjusted upon examination by the Internal Revenue Service if used in a future period. No income tax returns are currently under examination by taxing authorities.

On December 22, 2017, the U.S. government enacted H.R. 1, "An Act to provide for reconciliation pursuant to titles II and V of the concurrent resolution on the budget for fiscal year 2018" ("Tax Act"). Effective January 1, 2018, the Tax Act provides for a new global intangible low-taxed income ("GILTI") provision. Under the GILTI provision, certain foreign subsidiary earnings in excess of an allowable return on the foreign subsidiary's tangible assets are included in U.S. taxable income. The Company has not recorded any deferred taxes for future GILTI inclusions as any future inclusions are expected to be treated as a period expense and offset by net operating loss carryforwards in the U.S.

Stock-Based Compensation

The Company measures and recognizes compensation expense for all stock-based payment awards made to employees, officers and directors based on the estimated fair values of the awards as of the grant date. The Company records the value of the portion of the award that is ultimately expected to vest as expense over the requisite service period. The Company also accounts for equity instruments issued to non-employees using a fair value approach under Accounting Standards Codification ("ASC") subtopic 505-50. The Company values equity instruments and stock options granted using the Black-Scholes option pricing model.

Basic and Diluted Net Loss per Share of Common Stock

The Company uses the two-class method to compute net loss per common share because the Company has issued securities, other than common stock, that contractually entitle the holders to participate in dividends and earnings of the Company. The two-class method requires earnings for the period to be allocated between common stock and participating securities based upon their respective rights to receive distributed and undistributed earnings. Holders of each series of the Company's convertible preferred stock and select warrants are entitled to participate in distributions, when and if declared by the board of directors, that are made to common stockholders and, as a result, are considered participating securities.

Segment and Geographic Information

Operating segments are defined as components of an enterprise (business activity from which it earns revenue and incurs expenses) for which discrete financial information is available and regularly reviewed by the chief operating decision maker ("CODM") in deciding how to allocate resources and in assessing performance. The Company's CODM is its Chief Executive Officer. The Company views its operations and manages its business as a single operating and reporting segment. The Company holds assets in both the United States and Europe as of December 31, 2023 and 2022.

Foreign currency

Assets and liabilities are translated into the reporting currency using the exchange rates in effect on the consolidated balance sheet dates. Equity accounts are translated at historical rates, except for the change in retained earnings during the year, which is the result of the income statement translation process. Revenue and expense accounts are translated using the weighted average exchange rate during the period. The cumulative translation adjustments associated with the net assets of foreign subsidiaries are recorded in accumulated other comprehensive income/loss in the accompanying consolidated statements of stockholders' equity.

Warrants

The Company accounts for warrants as either equity-classified or liability-classified instruments based on an assessment of the warrant's specific terms and applicable authoritative guidance in FASB ASC Topic 480, Distinguishing Liabilities from Equity (ASC 480) and FASB ASC Topic 815, Derivatives and Hedging (ASC 815). The assessment considers whether the warrants are freestanding financial instruments pursuant to ASC 480, meet the definition of a liability pursuant to ASC 480, and whether the warrants meet all of the requirements for equity classification under ASC 815, including whether the warrants are indexed to our own stock and whether the warrant holders could potentially require "net cash settlement" in a circumstance outside of the company's control, among other conditions for equity classification. This assessment, which requires the use of professional judgment, is conducted at the time of warrant issuance and as of each subsequent quarterly period end date while the warrants are outstanding.

For warrants that meet all criteria for equity classification, the warrants are required to be recorded as a component of additional paid-in capital, on the consolidated statement of stockholders' deficit at the time of issuance. For warrants that do not meet all the criteria for equity classification, the warrants are required to be recorded at their initial fair value on the date of issuance, and on each balance sheet date thereafter. Changes in the estimated fair value of the warrants are recognized as a non-cash gain or loss in other expense, net, on the consolidated statement of operations. The fair value of the warrants was estimated using the Black-Scholes option pricing model.

C. Restatement of Previously Issued Consolidated Financial Statements

In connection with the preparation of the Company's consolidated financial statements as of and for the fiscal year ended December 31, 2023, the Company discovered that in the prior years it had not appropriately accounted for the liability associated with warrants to purchase the Company's common stock. The error resulted in an understatement of accumulated deficit of approximately $59.5 million, additional paid in capital of approximately $34.5 million, derivative and warrant liabilities of $10.2 million, an understatement of the fair value adjustment of derivative and warrant liability of $14.8 million, and an understatement in total other income of approximately $14.8 million, respectively, for the year ended December 31, 2022.

The misstatements were material to the previously issued financial statements of the Company and as a result, the Company has restated its consolidated balance sheet, consolidated statement of operations, consolidated statement of stockholder's equity, and consolidated statement of cash flows as of and for the fiscal year ended December 31, 2022, presented herein. The restatement includes adjustments to additional paid-in capital, accumulated deficit, derivative and warrant liabilities, fair value adjustment related to derivative and warrant liability, loss before income taxes, net loss, and net loss per share. The adjustments did not impact other comprehensive income for the year ended December 31, 2022. Therefore, the impact of the adjustments results in a decrease to comprehensive loss of $14.8 million (equal to the impact of adjustments on net loss in the table below), and the restated comprehensive loss is $26.6 million (previously reported as $41.4 million). The impact of the correction of the misstatements is summarized below. The Company has also corrected other errors which impacted various financial statement line items for the year ended December 31, 2022, as summarized below. Such additional corrections were not material individually or in the aggregate.

	As of December 31, 2022		
CORRECTED CONSOLIDATED BALANCE SHEET	**As previously reported**	**Impact of Adjustments**	**As Restated**
Prepaid expenses and other current assets	$ 1,877	$ (189)	$ 1,688
Total current assets	93,023	(189)	92,834
Total assets	115,529	(189)	115,340
Other current liabilities	422	297	719
Total current liabilities	11,726	297	12,023
Derivative and warrant liability	1	10,201	10,202
Total liabilities	29,722	10,498	40,220
Additional paid-in capital	401,799	34,470	436,269
Accumulated deficit	(308,572)	(45,157)	(353,729)
Total stockholder's equity	85,807	(10,687)	75,120
Total liabilities and stockholder's equity	115,529	(189)	115,340

	For the Year Ended December 31, 2022		
CORRECTED CONSOLIDATED STATEMENT OF OPERATIONS	**As previously reported**	**Impact of Adjustments**	**As Restated**
Revenue, net	$ 10,458	$ (297)	$ 10,161
Operating expenses:			
Cost of revenue	343	(121)	222
Research and development	19,614	189	19,803
Selling, general and administrative	15,343	(305)	15,038
Total operating expenses	52,963	(237)	52,726
Loss from operations	(42,505)	(60)	(42,565)
Fair value adjustment related to derivative and warrant liability	328	14,831	15,159
Interest and other income, net	760	753	1,513
Total other income	176	15,584	15,760
Loss before income taxes	(42,329)	15,524	(26,805)
Income tax (expense) benefit	786	(753)	33
Net loss	$ (41,543)	$ 14,771	$ (26,772)
Basic and diluted net loss per share of common stock:			
Net loss	$ (1.20)	$ 0.42	$ (0.78)

	For the Year Ended December 31, 2022		
CORRECTED CONSOLIDATED STATEMENT OF CASH FLOWS	**As previously reported**	**Impact of Adjustments**	**As Restated**
Cash flows from operating activities:			
Net (loss) income	$ (41,543)	$ 14,771	$ (26,772)
Adjustments to reconcile net loss to net cash used in operating activities:			
Change in fair value of derivative and warrant liability	(328)	(14,831)	(15,159)
Change in assets and liabilities:			
Prepaid expenses and other current assets	(657)	(237)	(894)
Other liabilities	(371)	297	(74)

	Additional Paid-In Capital			Accumulated Deficit		
CORRECTED STATEMENT OF STOCKHOLDERS' EQUITY	**As previously reported**	**Impact of Adjustments**	**As Restated**	**As previously reported**	**Impact of Adjustments**	**As Restated**
Balance at December 31, 2021	$ 396,957	$ 34,470	$ 431,427	$ (267,029)	$ (59,928)	$ (326,957)
Net loss	—	—	—	(41,543)	14,771	(26,772)
Balance at December 31, 2022	401,799	34,470	436,269	(308,572)	(45,157)	(353,729)

All referenced amounts for prior periods in these financial statements and the notes herein reflect the balances and amounts on a restated basis.

Restatement of Interim Financial Information (Unaudited)

Due to the misstatements described above, the Company has restated its unaudited condensed consolidated balance sheets, condensed consolidated statements of stockholder's equity, condensed consolidated statements of operations, and condensed consolidated statement of cash flows for the quarterly periods ended March 31, 2022, June 30, 2022, September 30, 2022, March 31, 2023, June 30, 2023, and September 30, 2023. The adjustments did not impact other comprehensive loss for any of the periods presented. Therefore, the impact of the adjustments on comprehensive loss equals the impact of adjustments on net loss for each period as detailed in the tables below.

Corrected Condensed Consolidated Balance Sheets (UNAUDITED)	March 31, 2023			March 31, 2022		
	As previously reported	Impact of Adjustments	As Restated	As previously reported	Impact of Adjustments	As Restated
Assets						
Current assets:						
Cash and cash equivalents	$ 40,181	$ —	$ 40,181	$ 100,242	$ —	$ 100,242
Securities at fair value	34,403	—	34,403	—	—	—
Short-term investments - other	20,700	—	20,700	1,338	—	1,338
Accounts and other receivables	7,822	—	7,822	3,320	—	3,320
Prepaid expenses and other current assets	1,174	—	1,174	880	(426)	454
Total current assets	104,280	—	104,280	105,780	(426)	105,354
Inventories	620	—	620	—	—	—
Property and equipment, net	744	—	744	835	—	835
Operating lease right-of-use assets	898	—	898	1,090	—	1,090
Long-term investments - other	—	—	—	17,564	—	17,564
Other long-term assets	53	—	53	437	—	437
Total assets	$ 106,595	$ —	$ 106,595	$ 125,706	$ (426)	$ 125,280
Liabilities and stockholders' equity						
Current liabilities:						
Accounts payable and accrued expenses	$ 10,098	$ 393	$ 10,491	$ 2,582	$ —	$ 2,582
Current portion of operating lease liabilities	470	—	470	356	—	356
Current portion of discount and rebate liabilities	4,746	—	4,746	—	—	—
Other current liabilities	302	—	302	7	—	7
Total current liabilities	15,616	393	16,009	2,945	—	2,945
Line of credit payable	12,914	—	12,914	—	—	—
Derivative and warrant liability	3	11,744	11,747	89	12,682	12,771
Operating lease liabilities, less current portion	736	—	736	1,144	—	1,144
Discount and rebate liabilities, less current portion	5,764	—	5,764	—	—	—
Other long-term liabilities	158	—	158	29	—	29
Total liabilities	35,191	12,137	47,328	4,207	12,682	16,889
Commitments and contingencies (Note D)						
Stockholders’ equity:						
Preferred stock:						
Undesignated preferred stock, $0.0001 par value, 10,000,000 shares authorized, no shares issued or outstanding as of March 31, 2023 or December 31, 2022	—	—	—	—	—	—
Common stock, $0.0001 par value, 250,000,000 shares authorized, 35,457,496 shares issued and 33,881,804 shares outstanding as of March 31, 2023; 35,450,257 shares issued and 34,540,304 shares outstanding as of December 31, 2022	3	—	3	3	—	3
Additional paid-in capital	402,786	34,470	437,256	397,925	34,470	432,395
Treasury stock, at cost	(10,983)	—	(10,983)	(7,536)	—	(7,536)
Accumulated deficit	(320,339)	(46,607)	(366,946)	(268,893)	(47,578)	(316,471)
Accumulated other comprehensive (loss) income	(63)	—	(63)	—	—	—
Total stockholders' equity	71,404	(12,137)	59,267	121,499	(13,108)	108,391
Total liabilities and stockholders' equity	$ 106,595	$ —	$ 106,595	$ 125,706	$ (426)	$ 125,280

Corrected Condensed Consolidated Balance Sheets (UNAUDITED)	June 30, 2023			June 30, 2022		
	As previously reported	Impact of Adjustments	As Restated	As previously reported	Impact of Adjustments	As Restated
Assets						
Current assets:						
Cash and cash equivalents	$ 66,196	$ —	$ 66,196	$ 76,779	$ —	$ 76,779
Securities at fair value	20,696	—	20,696	—	—	—
Short-term investments - other	479	—	479	4,199	—	4,199
Accounts and other receivables	14,033	—	14,033	2,820	—	2,820
Prepaid expenses and other current assets	2,023	—	2,023	3,637	(426)	3,211
Total current assets	103,427	—	103,427	87,435	(426)	87,009
Inventories	546	—	546	779	—	779
Property and equipment, net	689	—	689	904	—	904
Operating lease right-of-use assets	803	—	803	1,165	—	1,165
Long-term investments - other	—	—	—	33,535	—	33,535
Other long-term assets	53	—	53	440	—	440
Total assets	$ 105,518	$ —	$ 105,518	$ 124,258	$ (426)	$ 123,832
Liabilities and stockholders' equity						
Current liabilities:						
Accounts payable and accrued expenses	$ 10,510	$ —	$ 10,510	$ 3,600	$ —	$ 3,600
Current portion of capital lease obligation	—	—	—	469	—	469
Current portion of operating lease liabilities	456	—	456	—	—	—
Current portion of discount and rebate liabilities	6,965	—	6,965	1,796	—	1,796
Other current liabilities	321	—	321	1,294	48	1,342
Total current liabilities	18,252	—	18,252	7,159	48	7,207
Line of credit payable	12,709	—	12,709	12,800	—	12,800
Derivative and warrant liability	—	9,624	9,624	57	10,562	10,619
Operating lease liabilities, less current portion	627	—	627	1,082	—	1,082
Discount and rebate liabilities, less current portion	5,114	—	5,114	3,900	—	3,900
Other long-term liabilities	319	—	319	27	—	27
Total liabilities	37,021	9,624	46,645	25,025	10,610	35,635
Commitments and contingencies (Note D)						
Stockholders' equity:						
Preferred stock:						
Undesignated preferred stock, $0.0001 par value, 10,000,000 shares authorized, no shares issued or outstanding as of June 30, 2023 or December 31, 2022	—	—	—	—	—	—
Common stock, $0.0001 par value, 250,000,000 shares authorized, 35,503,697 shares issued and 33,928,005 shares outstanding as of June 30, 2023; 35,450,257 shares issued and 34,540,304 shares outstanding as of December 31, 2022	3	—	3	3	—	3
Additional paid-in capital	405,127	34,470	439,597	399,701	34,470	434,171
Treasury stock, at cost	(10,983)	—	(10,983)	(7,536)	—	(7,536)
Accumulated deficit	(325,425)	(44,094)	(369,519)	(292,935)	(45,506)	(338,441)
Accumulated other comprehensive (loss) income	(225)	—	(225)	—	—	—
Total stockholders' equity	68,497	(9,624)	58,873	99,233	(11,036)	88,197
Total liabilities and stockholders' equity	$ 105,518	$ —	$ 105,518	$ 124,258	$ (426)	$ 123,832

Corrected Condensed Consolidated Balance Sheets (UNAUDITED)	September 30, 2023			September 30, 2022		
	As previously reported	Impact of Adjustments	As Restated	As previously reported	Impact of Adjustments	As Restated
Assets						
Current assets:						
Cash and cash equivalents	$ 43,269	$ —	$ 43,269	$ 70,059	$ —	$ 70,059
Securities at fair value	39,672	—	39,672	—	—	—
Secured corporate notes	41,999	—	41,999	—	—	—
Short-term investments - other	485	—	485	5,832	—	5,832
Accounts and other receivables	9,927	—	9,927	6,583	—	6,583
Prepaid expenses and other current assets	1,661	—	1,661	2,659	(426)	2,233
Total current assets	137,013	—	137,013	85,133	(426)	84,707
Inventories	481	—	481	596	—	596
Property and equipment, net	642	—	642	852	—	852
Operating lease right-of-use assets	698	—	698	1,068	—	1,068
Long-term investments - other	—	—	—	31,463	—	31,463
Other long-term assets	148	—	148	439	—	439
Total assets	$ 138,982	$ —	$ 138,982	$ 119,551	$ (426)	$ 119,125
Liabilities and stockholders' equity						
Current liabilities:						
Accounts payable and accrued expenses	$ 13,080	$ —	$ 13,080	$ 4,279	$ —	$ 4,279
Current portion of operating lease liabilities	433	—	433	474	—	474
Current portion of discount and rebate liabilities	7,890	—	7,890	2,825	171	2,996
Other current liabilities	311	—	311	853	—	853
Total current liabilities	21,714	—	21,714	8,431	171	8,602
Line of credit payable	38,801	—	38,801	12,800	—	12,800
Derivative and warrant liability	—	5,948	5,948	35	16,139	16,174
Secured promissory note	5,073	—	5,073	—	—	—
Operating lease liabilities, less current portion	517	—	517	956	—	956
Discount and rebate liabilities, less current portion	4,987	—	4,987	3,509	—	3,509
Other long-term liabilities	420	—	420	26	—	26
Total liabilities	71,512	5,948	77,460	25,757	16,310	42,067
Commitments and contingencies (Note D)						
Stockholders' equity:						
Preferred stock:						
Undesignated preferred stock, $0.0001 par value, 10,000,000 shares authorized, no shares issued or outstanding as of September 30, 2023 or December 31, 2022	—	—	—	—	—	—
Common stock, $0.0001 par value, 250,000,000 shares authorized, 37,787,402 shares issued and 36,211,710 shares outstanding as of September 30, 2023; 35,450,257 shares issued and 34,540,304 shares outstanding as of December 31, 2022	3	—	3	3	—	3
Additional paid-in capital	418,138	34,470	452,608	400,677	34,470	435,147
Treasury stock, at cost	(10,983)	—	(10,983)	(7,536)	—	(7,536)
Accumulated deficit	(339,468)	(40,418)	(379,886)	(299,551)	(51,206)	(350,757)
Accumulated other comprehensive (loss) income	(220)	—	(220)	201	—	201
Total stockholders' equity	67,470	(5,948)	61,522	93,794	(16,736)	77,058
Total liabilities and stockholders' equity	$ 138,982	$ —	$ 138,982	$ 119,551	$ (426)	$ 119,125

Condensed consolidated statements of operations (UNAUDITED)	Three months ended March 31, 2023 As previously reported	2023 Impact of Adjustments	2023 As Restated	2022 As previously reported	2022 Impact of Adjustments	2022 As Restated
Revenue, net	$ 2,879	$ 297	$ 3,176	$ 3,965	$ —	$ 3,965
Operating expenses:						
Cost of revenue	125	—	125	8	—	8
Research and development	8,844	(189)	8,655	3,082	—	3,082
Selling, general and administrative	6,834	393	7,227	2,734	—	2,734
Acquired In-process research and development	—	—	—	—	—	—
Severance expense	—	—	—	—	—	—
Total operating expenses	15,803	204	16,007	5,824	—	5,824
Loss from operations	(12,924)	93	(12,831)	(1,859)	—	(1,859)
Other (expense) income:						
Interest expense	(182)	—	(182)	(5)	—	(5)
Fair value adjustment related to derivative and warrant liability	(2)	(1,543)	(1,545)	241	12,350	12,591
Fair value adjustment related to investments	196	—	196	(352)	—	(352)
Interest and other income, net	1,042	—	1,042	107	—	107
Total other (expense) income	1,054	(1,543)	(489)	(9)	12,350	12,341
Loss before income taxes	(11,870)	(1,450)	(13,320)	(1,868)	12,350	10,482
Income tax (expense) benefit	103	—	103	4	—	4
Net (loss) income	$ (11,767)	$ (1,450)	$ (13,217)	$ (1,864)	$ 12,350	$ 10,486
Basic and diluted net loss per share of common stock:						
Net (loss) income	$ (0.34)	$ (0.04)	$ (0.38)	$ (0.05)	$ 0.35	$ 0.30
Weighted average number of shares of common stock outstanding:						
Basic and diluted	34,466,542		34,466,542	34,506,597		34,506,597

	Three months ended June 30,						Six months ended June 30,					
	2023	2023	2023	2022	2022	2022	2023	2023	2023	2022	2022	2022
	As previously reported	Impact of Adjustments	As Restated	As previously reported	Impact of Adjustments	As Restated	As previously reported	Impact of Adjustments	As Restated	As previously reported	Impact of Adjustments	As Restated
Revenue, net	$ 8,470	$ —	$ 8,470	$ 1,300	$ (48)	$ 1,252	$ 11,349	$ 297	$ 11,646	$ 5,265	$ (48)	$ 5,217
Operating expenses:												
Cost of revenue	677	—	677	51	(20)	31	802	—	802	59	(20)	39
Research and development	7,433	—	7,433	4,795	—	4,795	16,277	(189)	16,088	7,877	—	7,877
Selling, general and administrative	7,005	(393)	6,612	3,558	20	3,578	13,839	—	13,839	6,292	20	6,312
Acquired In-process research and development	—	—	—	17,663	—	17,663	—	—	—	17,663	—	17,663
Total operating expenses	15,115	(393)	14,722	26,067	—	26,067	30,918	(189)	30,729	31,891	—	31,891
Loss from operations	(6,645)	(393)	(6,252)	(24,767)	(48)	(24,815)	(19,569)	486	(19,083)	(26,626)	(48)	(26,674)
Other (expense) income:												
Interest expense	(197)	—	(197)	(36)	—	(36)	(379)	—	(379)	(41)	—	(41)
Fair value adjustment related to derivative and warrant liability	—	2,118	2,118	32	2,120	2,152	—	575	575	273	14,470	14,743
Fair value adjustment related to investments	131	—	131	(352)	—	(352)	327	—	327	(495)	—	(495)
Interest and other income, net	1,553	—	1,553	366	753	1,119	2,593	—	2,593	264	753	1,017
Total other (expense) income	1,487	2,118	3,605	10	2,873	2,883	2,541	575	3,116	1	15,223	15,224
Loss before income taxes	(5,158)	2,511	(2,647)	(24,757)	2,825	(21,932)	(17,028)	1,061	(15,967)	(26,625)	15,175	(11,450)
Income tax (expense) benefit	74		74	715	(753)	(38)	177	—	177	719	(753)	(34)
Net (loss) income	$ (5,084)	$ 2,511	$ (2,573)	$ (24,042)	$ 2,072	$ (21,970)	$ (16,851)	$ 1,061	$ (15,790)	$ (25,906)	$ 14,422	$ (11,484)
Basic and diluted net loss per share of common stock:												
Net (loss) income	$ (0.15)	$ 0.07	$ (0.08)	$ (0.70)	0.06	$ (0.64)	$ (0.49)	0.03	$ (0.46)	$ (0.75)	0.42	$ (0.33)
Weighted average number of shares of common stock outstanding:												
Basic and diluted	33,898,233	—	33,898,233	34,447,206	—	34,447,206	34,180,818	—	34,180,818	34,476,737	—	34,476,737

	Three months ended September 30,						Nine months ended September 30,					
	2023	2023	2023	2022	2022	2022	2023	2023	2023	2022	2022	2022
	As previously reported	Impact of Adjustments	As Restated	As previously reported	Impact of Adjustments	As Restated	As previously reported	Impact of Adjustments	As Restated	As previously reported	Impact of Adjustments	As Restated
Revenue, net	$ 2,895	—	$ 2,895	$ 2,874	$ (123)	$ 2,751	$ 14,244	$ 297	$ 14,541	$ 8,139	$ (171)	$ 7,968
Operating expenses:												
Cost of revenue	144	—	144	141	(50)	91	946	—	946	200	(70)	130
Research and development	12,297	—	12,297	5,385	—	5,385	28,574	(189)	28,385	13,262	—	13,262
Selling, general and administrative	5,818	—	5,818	3,974	50	4,024	19,657	—	19,657	10,266	70	10,336
Acquired in-process research and development	—	—	—	—	—	—	—	—	—	17,663	—	17,663
Total operating expenses	18,259	—	18,259	9,500	—	9,500	49,177	(189)	48,988	41,391	—	41,391
Loss from operations	(15,364)	—	(15,364)	(6,626)	(123)	(6,749)	(34,933)	486	(34,447)	(33,252)	(171)	(33,423)
Other income (expense):												
Interest expense	(366)	—	(366)	(124)	—	(124)	(745)	—	(745)	(165)	—	(165)
Fair value adjustment related to derivative and warrant liability	—	3,678	3,678	22	(5,577)	(5,555)	—	4,253	4,253	295	8,893	9,188
Fair value adjustment related to investments	124	—	124	(139)	—	(139)	451	—	451	(634)	—	(634)
Interest and other income, net	1,738	—	1,738	218	—	218	4,331	—	4,331	482	753	1,235
Total other income (expense)	1,496	3,678	5,174	(23)	(5,577)	(5,600)	4,037	4,253	8,290	(22)	9,646	9,624
Loss before income taxes	(13,868)	3,678	(10,190)	(6,649)	(5,700)	(12,349)	(30,896)	4,739	(26,157)	(33,274)	9,475	(23,799)
Income tax (expense) benefit	(177)	—	(177)	33	—	33	—		—	752	(753)	(1)
Net loss	$ (14,045)	$ 3,678	$ (10,367)	$ (6,616)	$ (5,700)	$ (12,316)	$ (30,896)	$ 4,739	$ (26,157)	$ (32,522)	$ 8,722	$ (23,800)
Basic and diluted net loss per share of common stock:												
Net (loss) income	$ (0.40)	$ 0.10	$ (0.30)	$ (0.19)	$ (0.17)	$ (0.36)	$ (0.90)	$ 0.14	$ (0.76)	$ (0.94)	$ 0.25	$ (0.69)
Weighted average number of shares of common stock outstanding:												
Basic and diluted	34,724,614	—	34,724,614	34,494,702	—	34,494,702	34,364,075	—	34,364,075	34,482,791	—	34,482,791

Condensed Consolidated Statements of Changes in Stockholders' Equity (UNAUDITED)	Common Stock	Additional Paid-in Capital (As Restated)	Treasury Stock	Accumulated Deficit (As Restated)	Other Comprehensive Income (Loss)	Total Stockholders' (Deficit) Equity (As Restated)
Balance December 31, 2021 (As Reported)	$ 4	$ 396,957	$ (2,814)	$ (267,029)	$ —	$ 127,118
Effect of Restatement	—	34,470	—	(59,928)	—	(25,458)
Balance December 31, 2021 (As Restated)	$ 4	$ 431,427	$ (2,814)	$ (326,957)	$ —	$ 101,660
Net loss (As Restated)	—	—	—	10,486	—	10,486
Stock-based compensation expense	—	918	—	—	—	918
Shares repurchased as part of the Share Repurchase Program	(1)	—	(4,722)	—	—	(4,723)
Issuance of common stock in exchange for consulting services	—	50	—	—	—	50
Balance as of March 31, 2022 (As Restated)	$ 3	$ 432,395	$ (7,536)	$ (316,471)	$ —	$ 108,391
Net loss (As Restated)	—	—	—	(21,970)	—	(21,970)
Stock-based compensation expense	—	1,510	—	—	—	1,510
Issuance of common stock in exchange for consulting services	—	50	—	—	—	50
Issuance of common stock as part of the Employee Stock Purchase Plan	—	216	—	—	—	216
Balance as of June 30, 2022 (As Restated)	$ 3	$ 434,171	$ (7,536)	$ (338,441)	$ —	$ 88,197
Net loss (As Restated)	—	—	—	(12,316)	—	(12,316)
Stock-based compensation expense	—	911	—	—	—	911
Issuance of common stock in exchange for consulting services	—	65	—	—	—	65
Other comprehensive income	—	—	—	—	201	201
Balance as of September 30, 2022 (As Restated)	$ 3	$ 435,147	$ (7,536)	$ (350,757)	$ 201	$ 77,058
Balance December 31, 2022 (As Reported)	$ 3	$ 401,799	$ (7,536)	$ (308,572)	$ 113	$ 85,807
Effect of Restatement	—	34,470	—	(45,157)	—	(10,687)
Balance December 31, 2022 (As Restated)	$ 3	$ 436,269	$ (7,536)	$ (353,729)	$ 113	$ 75,120
Net loss (As Restated)	—	—	—	(13,217)	—	(13,217)
Stock-based compensation expense	—	591	—	—	—	591
Shares repurchased as part of the Share Repurchase Program	—	—	(3,447)	—	—	(3,447)
Issuance of common stock in exchange for consulting services	—	42	—	—	—	42
Severance expense	—	354	—	—	—	354
Other comprehensive loss	—	—	—	—	(176)	(176)
Balance as of March 31, 2023 (As Restated)	$ 3	$ 437,256	$ (10,983)	$ (366,946)	$ (63)	$ 59,267
Net loss (As Restated)	—	—	—	(2,573)	—	(2,573)
Stock-based compensation expense	—	1,103	—	—	—	1,103
Issuance of common stock in exchange for consulting services	—	25	—	—	—	25
Severance expense	—	1,048	—	—	—	1,048
Issuance of common stock as part of the Employee Stock Purchase Plan	—	165	—	—	—	165
Other comprehensive loss	—	—	—	—	(162)	(162)
Balance as of June 30, 2023 (As Restated)	$ 3	$ 439,597	$ (10,983)	$ (369,519)	$ (225)	$ 58,873
Net loss (As Restated)	—	—	—	(10,367)	—	(10,365)
Stock-based compensation expense	—	1,387	—	—	—	1,387
Issuance of common stock in connection with the Proposed Merger (Note K)	—	11,500	—	—	—	11,500
Issuance of common stock in exchange for consulting services	—	71	—	—	—	71
Severance expense	—	53	—	—	—	53
Other comprehensive loss	—	—	—	—	5	5
Balance as of September 30, 2023	$ 3	$ 452,608	$ (10,983)	$ (379,886)	$ (220)	$ 61,524

Condensed Consolidated Statements of Cash Flows (UNAUDITED)	Three Months Ended March 31,					
	2023			2022		
	As Reported	Impact of Adjustments	As Restated	As Reported	Impact of Adjustments	As Restated
Cash flows from operating activities:						
Net income (loss)	$ (11,767)	$ (1,450)	$ (13,217)	$ (1,864)	$ 12,350	$ 10,486
Adjustments to reconcile net loss to net cash used in operating activities:						
Stock-based compensation expense	591	—	591	918	—	918
Severance expense	354	—	354	—	—	—
Depreciation and amortization expense	79	—	79	65	—	65
Fair value adjustment related to derivative and warrant liability	2	1,543	1,545	(241)	(12,350)	(12,591)
Fair value adjustment related to investments	(196)	—	(196)	352	—	352
Consulting fees paid in common stock	42	—	42	50	—	50
Gain on foreign currency exchange rates	(240)	—	(240)	—	—	—
Change in assets and liabilities:						
Accounts and other receivables	477	—	477	(1,792)	—	(1,792)
Prepaid expenses and other current assets	703	—	703	303	—	303
Inventories	51	—	51	—	—	—
Operating lease right-of-use assets	80	—	80	51	—	51
Accounts payable and accrued expenses	3,929	(93)	3,836	(486)	—	(486)
Discount and rebate liabilities	1,528	—	1,528	—	—	—
Operating lease liabilities	(107)	—	(107)	(88)	—	(88)
Other liabilities	429	—	429	(821)	—	(821)
Net cash used in operating activities	(4,045)	—	(4,045)	(3,553)	—	(3,553)
Cash flows from investing activities:						
Purchases of property and equipment	(29)	—	(29)	(16)	—	(16)
Purchases of investments	(17,526)	—	(17,526)	(3,832)	—	(3,832)
Net cash used in investing activities	(17,555)	—	(17,555)	(3,848)	—	(3,848)
Cash flows from financing activities:						
Proceeds from issuance of debt	12,914	—	12,914	—	—	—
Payments of principal on insurance financing arrangements	(415)	—	(415)	—	—	—
Repayment of debt	(12,800)	—	(12,800)	—	—	—
Payment to repurchase shares as part of the Share Repurchase Program	(3,447)	—	(3,447)	(4,723)	—	(4,723)
Repayment of principal on finance lease liabilities	(2)	—	(2)	(10)	—	(10)
Net cash provided by financing activities	(3,750)	—	(3,750)	(4,733)	—	(4,733)
Effect of exchange rate changes on cash and cash equivalents	65	—	65	—	—	—
Net decrease in cash and cash equivalents	(25,285)	—	(25,285)	(12,134)	—	(12,134)
Cash and cash equivalents, beginning of period	65,466	—	65,466	112,346	—	112,346
Cash and cash equivalents, end of period	$ 40,181	—	$ 40,181	$ 100,212	—	$ 100,212
Supplemental cash flow information:						
Cash paid for interest	$ 68	—	$ 68	$ 5	—	$ 5

Condensed Consolidated Statements of Cash Flows (UNAUDITED)	Six Months Ended June 30,					
	2023			2022		
	As Reported	Impact of Adjustments	As Restated	As Reported	Impact of Adjustments	As Restated
Cash flows from operating activities:						
Net loss	$ (16,851)	$ 1,061	$ (15,790)	$ (25,906)	$ 14,422	$ (11,484)
Adjustments to reconcile net loss to net cash used in operating activities:						
Stock-based compensation expense	1,694	—	1,694	2,428	—	2,428
Severance expense	1,402	—	1,402	—	—	—
Depreciation and amortization expense	157	—	157	246	—	246
Fair value adjustment related to derivative and warrant liability	—	(481)	(481)	(273)	(14,470)	(14,743)
Fair value adjustment related to investments	(327)	—	(327)	495	—	495
Loss on sublease and disposal of property and equipment	—	—	—	9	—	9
Consulting fees paid in common stock	67	—	67	100	—	100
Acquired in-process research and development	—	—	—	17,663	—	17,663
Gain on foreign currency exchange rates	(138)	—	(138)	—	—	—
Change in assets and liabilities:						
Accounts and other receivables	(5,734)	—	(5,734)	(1,292)	—	(1,292)
Prepaid expenses and other current assets	(146)	(189)	(335)	(1,892)	—	(1,892)
Inventories	125	—	125	39	—	39
Operating lease right-of-use assets	161	—	161	(24)	—	(24)
Accounts payable and accrued expenses	3,338	(391)	2,947	630	—	630
Discount and rebate liabilities	3,097	—	3,097	496	—	496
Operating lease liabilities	(216)	—	(216)	(37)	—	(37)
Other liabilities	622	—	622	(339)	48	(291)
Net cash used in operating activities	(12,749)	—	(12,749)	(7,657)	—	(7,657)
Cash flows from investing activities:						
Acquisitions, net	—	—	—	(14,090)	—	(14,090)
Purchases of property and equipment	(52)	—	(52)	(31)	—	(31)
Purchases of investments	(17,467)	—	(17,467)	(23,832)	—	(23,832)
Maturities of long-term investments, net	34,000	—	34,000	1,025	—	1,025
Net cash used in investing activities	16,481	—	16,481	(36,928)	—	(36,928)
Cash flows from financing activities:						
Proceeds from issuance of debt	12,800	—	12,800	12,800	—	12,800
Proceeds from insurance financing arrangements	1,256	—	1,256	1,273	—	1,273
Proceeds from Employee Stock Purchase Plan	166	—	166	216	—	216
Payments of principal on insurance financing arrangements	(564)	—	(564)	(469)	—	(469)
Repayment of debt	(13,007)	—	(13,007)	—	—	—
Payment of offering costs	—	—	—	(68)	—	—
Payment to repurchase shares as part of the Share Repurchase Program	(3,447)	—	(3,447)	(4,723)	—	(4,723)
Repayment of principal on finance lease liabilities	(3)	—	(3)	(11)	—	(11)
Net cash provided by financing activities	(2,799)	—	(2,799)	9,018	—	9,086
Effect of exchange rate changes on cash and cash equivalents	(203)	—	(203)	—	—	—
Net decrease in cash and cash equivalents	730	—	730	(35,567)	—	(35,499)
Cash and cash equivalents, beginning of period	65,466	—	65,466	112,346	—	112,346
Cash and cash equivalents, end of period	$ 66,196	$ —	$ 66,196	$ 76,779	$ —	$ 76,847
Supplemental cash flow information:						
Cash paid for interest	$ 261	—	$261	$ 41	—	$ 41

Condensed Consolidated Statements of Cash Flows (UNAUDITED)	Nine Months Ended September 30, 2023 As Reported	2023 Impact of Adjustments	2023 As Restated	2022 As Reported	2022 Impact of Adjustments	2022 As Restated
Cash flows from operating activities:						
Net loss	$ (30,896)	$ 4,739	$ (26,157)	$ (32,522)	$ 8,722	$ (23,800)
Adjustments to reconcile net loss to net cash used in operating activities:						
Stock-based compensation expense	3,081	—	3,081	3,339	—	3,339
Severance expense	1,401	—	1,401	—	—	—
Depreciation and amortization expense	219	—	219	644	—	644
Fair value adjustment related to derivative and warrant liability	—	(4,159)	(4,159)	(295)	(8,893)	(9,188)
Fair value adjustment related to investments	(451)	—	(451)	634	—	634
Loss on sublease and disposal of property and equipment	157	—	157	9	—	9
Consulting fees paid in common stock	138	—	138	165	—	165
Acquired in-process research and development	—	—	—	17,663	—	17,663
Gain on foreign currency exchange rates	(30)	—	(30)	—	—	—
Change in assets and liabilities:						
Accounts and other receivables	(1,628)	—	(1,628)	(4,646)	—	(4,646)
Prepaid expenses and other current assets	216	(189)	27	(1,196)	—	(1,196)
Inventories	190	—	190	280	—	280
Operating lease right-of-use assets	243	—	243	82	—	82
Other long-term assets	(93)	—	(93)	-	—	—
Accounts payable and accrued expenses	5,791	(391)	5,400	1,262	—	1,262
Discount and rebate liabilities	3,895	—	3,895	858	—	858
Operating lease liabilities	(326)	—	(326)	(160)	—	(160)
Other liabilities	716	—	716	(372)	171	(201)
Net cash used in operating activities	(17,377)	—	(17,377)	(14,255)	—	(14,255)
Cash flows from investing activities:						
Acquisitions, net	—	—	—	(14,090)	—	(14,090)
Purchases of property and equipment	(224)	—	(224)	(59)	—	(59)
Purchases of investments	(45,821)	—	(45,821)	(23,832)	—	(23,832)
Purchases of Secured corporate notes	(25,426)	—	(25,426)	—	—	—
Maturities of long-term investments, net	43,496	—	43,496	1,325	—	1,325
Net cash used in investing activities	(27,975)	—	(27,975)	(36,656)	—	(36,656)
Cash flows from financing activities:						
Proceeds from issuance of debt	38,801	—	38,801	12,800	—	12,800
Proceeds from insurance financing arrangements	1,256	—	1,256	1,273	—	1,273
Proceeds from Employee Stock Purchase Plan	219	—	219	216	—	216
Payments of principal on insurance financing arrangements	(564)	—	(564)	(876)	—	(876)
Repayment of debt	(12,800)	—	(12,800)	—	—	—
Payment of offering costs	—	—	—	(68)	—	—
Payment to repurchase shares as part of the Share Repurchase Program	(3,447)	—	(3,447)	(4,723)	—	(4,723)
Repayment of principal on finance lease liabilities	(5)	—	(5)	(13)	—	(13)
Net cash provided by financing activities	23,460	—	23,460	8,609	—	8,677
Effect of exchange rate changes on cash and cash equivalents	(305)	—	(305)	15	—	15
Net decrease in cash and cash equivalents	(22,197)	—	(22,197)	(42,287)	—	(42,219)
Cash and cash equivalents, beginning of period	65,466	—	65,466	112,346	—	112,346
Cash and cash equivalents, end of period	$ 43,269	$ —	$ 43,269	$ 70,059	$ —	$ 70,127
Supplemental cash flow information:						
Cash paid for interest	$ 456	$ —	$ 456	$ 165	$ —	$ 165
Right-of-use assets obtained in exchange for lease liabilities	—	—	—	—	—	—
Supplemental disclosure of noncash investing activities:						
Issuance of common stock in connection with the Merger (Note K)	(11,500)	—	(11,500)	—	—	—
Supplemental disclosure of noncash financing activities:						
Issuance of secured promissory note for the Merger (Note K)	(5,073)	—	(5,073)	—	—	—

D. Accounts and Other Receivables

Accounts and other receivables consist of the following (in thousands):

	December 31, 2023	December 31, 2022
Accounts receivable	$ 16,119	$ 7,185
Other receivables	1,258	1,114
Total accounts and other receivables	$ 17,377	$ 8,299

E. Prepaid Expenses and Other Current Assets

Prepaid expenses and other current assets consist of the following (in thousands):

	December 31,	
	2023	2022
Prepaid insurance	$ 1,172	$ 577
Other prepaid expenses and current assets	652	1,111
Total prepaid expenses and other current assets	$ 1,824	$ 1,688

F. Property and Equipment

Property and equipment consists of the following (in thousands):

	December 31,	
	2023	2022
Laboratory equipment	$ 711	$ 710
Furniture and office equipment	157	104
Computers and hardware	643	462
Leasehold improvements	710	710
Finance lease right-of-use assets	149	251
Total property and equipment	2,370	2,237
Less: accumulated depreciation and amortization	(1,634)	(1,443)
Property and equipment, net	$ 736	$ 794

The estimated useful lives of property and equipment are as follows:

Asset Category	Useful Life (in years)
Laboratory equipment	10
Furniture and office equipment	5 - 10
Computers and hardware	3 - 7
Leasehold improvements	9

Depreciation and amortization expense, including amounts pertaining to assets held under finance leases, was approximately $245,000 and $257,000 for the years ended December 31, 2023 and 2022, respectively.

G. Accounts Payable and Accrued Expenses

Accounts payable and accrued expenses consist of the following (in thousands):

	December 31,	
	2023	2022
Accrued payroll	10,671	1,673
Accrued professional fees	675	256
Accounts payable	13,987	3,611
Other accrued expenses	3,070	629
Total accounts payable and accrued expenses	$ 28,403	$ 6,169

H. Debt Obligations

Secured Promissory Note

In connection with the Merger (Note R), on August 30, 2023, the Company and Nantahala, entered into a secured promissory note payable by Zevra to Nantahala in the original principal amount of $5.0 million (the "Nantahala Note"). The Nantahala Note will initially bear interest at 9.0% per annum, payable quarterly in arrears in cash. The interest rate will increase to 12.0% per annum if the Nantahala Note remains unpaid after six months from its issue date. The additional 3.0% interest will be paid in shares of Zevra's common stock based on the volume weighted average trading price ("VWAP") of Zevra's common stock during the twenty consecutive trading days ending on the date before such interest payment date. Beginning on the first interest payment date following the second anniversary of the Nantahala Note, and on each interest payment date thereafter, Zevra is required to make $0.6 million amortization payments on the Nantahala Note until it is paid in full. All principal and unpaid interest on the Nantahala Note is due on August 30, 2026, the third anniversary of the Nantahala Note, and therefore is included in long-term liabilities as of December 31, 2023. Zevra may prepay the Nantahala Note at any time without penalty. The Nantahala Note is secured by Zevra's interest in (i) the loan assets under the Loan Purchase Agreement described in Note R; (ii) the note assets under the Note Purchase Agreement described in Note R; (iii) the Bridge Loan described in Note R; and (iv) the proceeds therefrom. The Company used the proceeds from the Nantahala Note, along with $12.0 million in cash and 98,683 shares of Zevra's common stock, to acquire Acer's term loans, as more fully described in Note R.

As of December 31, 2023, the Company's secured promissory note outstanding, in the aggregate principal amount, was as follows (in thousands):

	December 31, 2023
Secured promissory note	$ 5,000
Unamortized original issue premium	148
Less: debt issuance costs	(82)
Secured promissory note, net	$ 5,066

Future minimum principal payments under the secured promissory note as of December 31, 2023, were as follows (in thousands):

Year Ending December 31, 2023	
2024	$ —
2025	1,200
2026	3,800
Total minimum payments	5,000
Plus: unamortized debt premium and debt issuance costs	66
Secured promissory note, net	$ 5,066

Line of Credit

On May 31, 2022, the Company and Ameris Bank, as lender, entered into a $20.0 million revolving loan agreement (the "Line of Credit"). Proceeds of the revolving facility provided by the Line of Credit are to be used for general corporate purposes. Loans under the Line of Credit bear interest at the Secured Overnight Financing Rate ("SOFR") plus 1.60%, with a SOFR floor of 0.00%.

The revolving facility under the Line of Credit is secured by a perfected security interest in deposit accounts. The revolving facility under the Line of Credit is subject to customary affirmative and negative covenants.

The latest maturity date of the loans under the Line of Credit is May 31, 2025, and therefore is included in long-term liabilities as of December 31, 2022. The Line of Credit contains customary events of default that could lead to an acceleration of the loans, including cross-default, bankruptcy and payment defaults. As of December 31, 2022, the Company had drawn $12.8 million from the Line of Credit to finance the transactions under the Arimoclomol Purchase Agreement, and this amount is supported by a $12.8 million certificate of deposit which is shown as long-term investments - other in the consolidated balance sheet. The remaining $7.2 million under the Line of Credit is in a separate interest-bearing certificate of deposit and is also recorded as long-term investments - other in the consolidated balance sheet as of December 31, 2022. These certificates of deposit were pledged as collateral against the Line of Credit and cannot be redeemed so long as the $20.0 million remains available under the Line of Credit. The total value of the certificates of deposit held with Ameris Banks must meet or exceed the amount available to borrow under the Line of Credit so long as the Line of Credit remains active. On January 31, 2023, the Company repaid the $12.8 million outstanding under the Line of Credit in full and subsequently closed the Line of Credit. In conjunction with closing the Line of Credit, the maturity dates of the certificates of deposit were modified to May 7, 2023.

On January 26, 2023, the Company and Wells Fargo, as lender, entered into a margin account agreement. The Company's investments are used as collateral for the loan and the amount the Company is able to borrow is limited to 80-90% of its outstanding investment balance held with Wells Fargo (the "Collateral Requirement"). The margin account bears interest at the Prime rate minus 225 basis-points. As of December 31, 2023, $37.7 million was outstanding under the margin account and the remaining borrowing capacity was approximately $6.4 million. The margin loan is included in current liabilities as of December 31, 2023, as the Company expects the outstanding balance to be due and payable in the coming year based on the anticipated level of investment balance that will be available over the course of fiscal year 2024 to meet the Collateral Requirement.

I. Commitments and Contingencies

Legal Matters

From time to time, the Company is involved in various legal proceedings arising in the normal course of business. For some matters, a liability is not probable, or the amount cannot be reasonably estimated and, therefore, an accrual has not been made. However, for such matters when it is probable that the Company has incurred a liability and can reasonably estimate the amount, the Company accrues and discloses such estimates.

APADAZ License Agreement

On May 31, 2023, the Company and KVK-Tech, Inc. ("KVK") terminated the Collaboration and License Agreement (the "Agreement") that the parties entered into on October 25, 2018. In conjunction with the termination of the Agreement, the Company agreed to pay a settlement to KVK of $0.9 million, which is included in research and development in the consolidated statement of operations for the year ended December 31, 2023, and was paid in October 2023.

Stockholder Litigation Related to the Merger

On October 12, 2023, Brodsky & Smith, purporting to act as counsel for Jerry Beavee, who was asserted to be a stockholder of Acer, filed a complaint entitled Jerry Beavee v. Acer Therapeutics Inc., et al., No. 1:23-cv-08995 in the United States District Court for the Southern District of New York alleging that defendants violated Section 14(a) and 20(a) of the Securities Exchange Act of 1934 by filing the Preliminary Merger Registration Statement which allegedly omitted certain information that such counsel asserts is material to the Acer's required disclosure. The complaint prays that, if asserted omissions are not adequately corrected, then Beavee will seek to enjoin Acer from holding a stockholder meeting to approve Merger and, if the Merger closes, will seek to rescind it and seek an award of damages. On October 17, 2023, the court set an initial pretrial conference for December 13, 2023.

On October 20, 2023, Long Law, LLC and Acocelli Law, PLLC, purporting to act as counsel for Kevin Turner, who was asserted to be a stockholder of Acer, filed a complaint entitled Kevin Turner v. Acer Therapeutics Inc., et al., No. 1:23-cv-01185 in the United States District Court for the District of Delaware alleging that defendants violated Section 14(a) and 20(a) of the Securities Exchange Act of 1934 as well as SEC Rule 14a-9 by filing the Definitive Proxy Statement which allegedly omitted certain information that such counsel asserts is material to Acer's required disclosure. The complaint prays that, if asserted omissions are not adequately corrected, then Turner will seek to enjoin Acer from holding a stockholder meeting to approve the Merger and, if the Merger closes, will seek to rescind it and seek an award of damages.

On October 20, 2023, Long Law, LLC, purporting to act as counsel for Matthew Jones, who was asserted to be a stockholder of Acer, filed a complaint entitled Matthew Jones v. Acer Therapeutics Inc., et al., No. 1:23-cv-01186 in the United States District Court for the District of Delaware alleging that defendants violated Section 14(a) and 20(a) of the Securities Exchange Act of 1934 as well as SEC Rule 14a-9 by filing the Definitive Proxy Statement which allegedly omitted certain information that such counsel asserts is material to the Acer's required disclosure. The complaint prays that, if asserted omissions are not adequately corrected, then Jones will seek to enjoin Acer from holding a stockholder meeting to approve the Merger and, if the Merger closes, will seek to rescind it and seek an award of damages.

As of December 31, 2023 and 2022, no accruals have been made related to commitments and contingencies. The Company intends to vigorously defend these lawsuits and believes that it has meritorious defenses to each. However, litigation is inherently uncertain, and any judgment or injunctive relief entered against the Company or any adverse settlement could adversely affect the Company's business, results of operations and financial condition.

J. Stock and Warrants

Authorized, Issued, and Outstanding Common Shares

As of December 31, 2023, and 2022, the Company had authorized shares of common stock of 250,000,000 shares. Of the authorized shares, 43,110,360 and 35,450,257 shares of common stock were issued as of December 31, 2023, and 2022, respectively, and 41,534,668 and 34,540,304 shares of common stock were outstanding as of December 31, 2023, and 2022, respectively.

As of December 31, 2023, and 2022, the Company had reserved authorized shares of common stock for future issuance as follows:

	December 31,	
	2023	**2022**
Outstanding awards under equity incentive plans	8,023,142	2,456,407
Outstanding common stock warrants	5,603,729	4,252,600
Possible future issuances under equity incentive plans	1,728,885	4,421,508
Possible future issuances under employee stock purchase plan	1,340,172	1,417,365
Total common shares reserved for future issuance	16,695,928	12,547,880

Common Stock Activity

The following table summarizes common stock activity for the years ended December 31, 2023, and 2022:

	Shares of Common Stock
Balance as of January 1, 2022	35,005,640
Common stock issued as compensation to third-parties	41,821
Common stock repurchased as a result of the Stock Repurchase Program	(589,792)
Common stock issued as a result of the Employee Stock Purchase Plan	82,635
Balance as of December 31, 2022	34,540,304
Common stock issued as compensation to third-parties	44,791
Common stock issued as a result of stock warrants exercised	110
Common stock issued as a result of stock options exercised	7,500
Common stock repurchased as a result of the Stock Repurchase Program	(665,739)
Common stock issued in connection with the Merger (Note R)	7,530,509
Common stock issued as a result of the Employee Stock Purchase Plan	77,193
Balance as of December 31, 2023	41,534,668

Authorized, Issued, and Outstanding Preferred Stock

As of December 31, 2023, and 2022, the Company had 10,000,000 shares of authorized, unallocated and unissued preferred stock. As of December 31, 2023, and 2022, no shares of preferred stock were designated, issued or outstanding.

Warrants to Purchase Common Stock

In prior periods, the Company issued warrants to purchase common stock to various third parties, of which 4,221,240 remain outstanding as of December 31, 2023, and are immediately exercisable. Of the outstanding and exercisable warrants, 120,192 qualify as participating securities under ASC Topic 260, *Earnings per Share*, and are treated as such in the net loss per share calculation (Note N). The Company may be required to redeem these warrants for a cash amount equal to the Black-Scholes value of the portion of the warrants to be redeemed (the "Put Option").

In connection with the Merger (Note R), in November 2023, the Company directly issued to certain investors an aggregate of 1,382,489 shares of its common stock, par value $0.0001 per share, and accompanying warrants to purchase up to 1,382,489 shares of its common stock (the "2023 Warrants") at a combined offering price of $4.34 per share of common stock and the Warrants and an aggregate of 917,934 shares of its common stock in exchange for the cancellation of a warrant to purchase 2,920,306 shares of common stock of Acer. The Warrants are immediately exercisable and expire on November 22, 2028. The Company intends to use the net proceeds of approximately $6.0 million from the offering for general corporate purposes. These warrants are separately exercisable by the warrant holders. While the warrants are outstanding (but unexercised), the warrant holders will participate in any dividend or other distribution of the Company's assets to its common stockholders by way of return of capital or otherwise. As of December 31, 2023, none of the warrants have been exercised. The warrants have been evaluated to determine the appropriate accounting and classification pursuant to ASC Topic 480, Distinguishing Liabilities from Equity, and ASC Topic 815, Derivatives and Hedging. Generally, freestanding warrants should be classified as (i) liabilities if the warrant terms allow settlement of the warrant exercise in cash and (ii) equity if the warrant terms only allow settlement in shares of common stock. Based on the terms of the warrants issued in November 2023, management concluded that they should be classified as a liability with subsequent remeasurement to fair value as of the end of each reporting period. The Company recorded a $4.5 million liability at inception.

Based on the conclusion reached regarding the liability classification of the 2023 Warrants, management re-evaluated the classification of the common stock warrants originally issued in 2021 ("2021 Warrants"), of which 4.1 million remain outstanding as of December 31, 2022, and 2023. As a result of this analysis, management identified a settlement term that was consistently present in both the 2021 Warrants and the 2023 Warrants, resulting in a conclusion that the 2021 Warrants should have originally been classified as a liability at inception with subsequent remeasurement to fair value as of the end of each reporting period. This resulted in a restatement of the consolidated financial statements as of and for the year ended December 31, 2022, as discussed in Note C.

As noted above, the Company determined that these warrants and the Put Option should be recorded as a liability and stated at fair value at each reporting period. Changes to the fair value of the warrant liability are recorded through the consolidated statements of operations as a fair value adjustment. As of December 31, 2023, and 2022, the fair value of the liability associated with these warrants and the Put Option was approximately $16.1 million and $10.2 million, respectively. The fair value adjustment related to these warrants and the Put Option was approximately $(1.4) million for the ended December 31, 2023, and approximately $14.8 million for the year ended December 31, 2022.

K. Stock-Based Compensation

The Company maintains a stock-based compensation plan (the "Incentive Stock Plan") that governs stock awards made to employees and directors prior to completion of the IPO.

In November 2014, the Board of Directors of the Company ("the Board"), and in April 2015, the Company's stockholders, approved the Company's 2014 Equity Incentive Plan (the "2014 Plan"), which became effective in April 2015. The 2014 Plan provides for the grant of stock options, other forms of equity compensation, and performance cash awards. In June 2021, the Company's stockholders approved an Amended and Restated 2014 Equity Incentive Plan (the "A&R 2014 Plan"), following its adoption by the Board in April 2021, which among other things added 4,900,000 shares to the maximum number of shares of common stock to be issued under the plan and extended the annual automatic increases (discussed further below) until January 1, 2031 and eliminated individual grant limits that applied under the 2014 Plan to awards that were intended to comply with the exemption for "performance-based compensation" under Code Section 162(m). The maximum number of shares of common stock that may be issued under the A&R 2014 Plan is 8,271,497 as of December 31, 2023. The number of shares of common stock reserved for issuance under the A&R 2014 Plan will automatically increase on January 1 of each year, beginning on January 1, 2016, and ending on and including January 1, 2031, by 4% of the total number of shares of the Company's capital stock outstanding on December 31 of the preceding calendar year, or a lesser number of shares determined by the Board. Pursuant to the terms of the A&R 2014 Plan, on January 1, 2024, the common stock reserved for issuance under the A&R 2014 Plan automatically increased by 1,661,386 shares.

During the year ended December 31, 2023, 7,500 stock options were exercised. During the year ended December 31,2022, no stock options were exercised.

In June 2021, the Company's stockholders approved an Employee Stock Purchase Plan (the "ESPP"), following its adoption by the Board in April 2021. The maximum number of shares of common stock that may be issued under the ESPP is 1,500,000. The first offering period under the ESPP began on October 1, 2021, and the first purchase date occurred on May 31, 2022. As of December 31, 2023, 159,828 shares have been issued under the ESPP.

In January 2023, our Board approved the 2023 Employment Inducement Award Plan (as amended, the "2023 Plan"). The maximum number of shares of common stock that may be issued under the 2023 Plan is 1,500,000. In February 2024, our Board approved an amendment to the 2023 Plan to increase the aggregate number of shares of common stock available for issuance under the 2023 Plan from 1,500,000 shares to 4,500,000 shares.

In May 2023, the Board approved the Ninth Amended and Restated Non-Employee Director Compensation Policy (the "Non-Employee Director Compensation Policy"). The equity compensation made pursuant to the Non-Employee Director Compensation Policy will be granted under the A&R 2014 Plan.

Stock-based compensation expense recorded under the Incentive Stock Plan, A&R 2014 Plan, ESPP, and 2023 Plan is included in the following line items in the accompanying consolidated statements of operations (in thousands):

	Year ended December 31,	
	2023	2022
Research and development	$ 2,664	$ 1,443
General and administrative	3,290	2,851
Total stock-based compensation expense	$ 5,954	$ 4,294

There was no stock-based compensation expense related to performance-based awards recognized during the year ended December 31, 2023. There was $0.4 million of stock-based compensation related to performance-based awards recognized during the year ended December 31, 2022.

As a result of the Mickle Transition Agreement and the Pascoe Transition Agreement, as further discussed in Note P, certain stock options were modified, resulting in a net decrease in stock-based compensation expense of $1.2 million for the year ended December 31, 2023. The effects of these modifications are reflected in the table above within selling, general and administrative expenses.

Stock Option Awards

The Company estimates the fair value of stock options using the Black-Scholes option-pricing model, which requires the use of subjective assumptions, including the expected term of the option, the expected stock price volatility, expected dividend yield and the risk-free interest rate for the expected term of the option. The expected term represents the period of time the stock options are expected to be outstanding. Due to the lack of sufficient historical exercise data to provide a reasonable basis upon which to otherwise estimate the expected term of the stock options, the Company uses the simplified method to estimate the expected term for its "plain vanilla" stock options. Under the simplified method, the expected term of an option is presumed to be the mid-point between the vesting date and the end of the contractual term. Some options, for example those that have exercise prices in excess of the fair value of the underlying stock, are not considered "plain vanilla" stock options. For these options, the Company uses an expected term equal to the contractual term of the option. Expected volatility is based on the Company's historical volatility over the estimated expected term of the stock options. The Company assumes no dividend yield because dividends are not expected to be paid in the near future, which is consistent with the Company's history of not paying dividends.

The Company recognizes compensation expense related to stock-based payment transactions upon satisfaction of the requisite service or vesting requirements. Forfeitures are estimated at the time of grant and revised based on actual forfeitures, if necessary, in subsequent periods if actual forfeitures differ from those estimates.

Using the Black-Scholes option-pricing model, the weighted-average fair value of awards granted during the years ended December 31, 2023, and 2022, fair value was $3.65 and $5.01 per share, respectively. The assumptions used to estimate fair value are as follows:

	Year Ended December 31,	
	2023	**2022**
Risk-free interest rate	3.34% - 4.79%	1.70% - 3.80%
Expected term (in years)	5.50 - 10.00	5.50 - 7.00
Expected volatility	89.48% - 93.67%	91.28% - 98.91%
Expected dividend yield	0	0

The activity under the Incentive Stock Plan, A&R 2014 Plan, and 2023 Plan for the year ended December 31, 2023 , is summarized as follows:

	Number of Options	**Weighted Average Exercise Price**	**Weighted Avg Remaining Contractual Term**	**Aggregate Intrinsic Value**
Outstanding balance at January 1, 2023	2,456,407	$ 16.84	8.51	$ 11,819
Granted	5,779,679	$ 4.68	—	—
Exercised or released	7,500	$ 4.67	—	—
Canceled or forfeited	205,444	$ 4.70	—	$ 122
Expired	—	—	—	—
Outstanding balance at December 31, 2023	8,023,142	$ 8.40	8.68	$ 11,089
Exercisable at December 31, 2023	2,408,165	$ 16.28	7.81	$ 1,778
Vested and expected to vest at December 31, 2023	6,972,858	$ 8.96	8.62	$ 9,112

Information regarding currently outstanding and exercisable options as of December 31, 2023, is as follows:

	Options Outstanding		**Options Exercisable**	
Exercise Price	**Number of Shares**	**Weighted Avg Remaining Contractual Term**	**Number of Shares**	**Weighted Avg Remaining Contractual Term**
$2.848 to $10.00	7,742,171	8.84	2,127,194	8.25
$10.01 to $30.00	69,375	7.17	69,375	7.17
$30.01 to $50.00	68,514	5.11	68,514	5.11
$50.01 to $70.00	41,866	3.08	41,866	3.08
$70.01 to $327.20	101,216	2.70	101,216	2.70
	8,023,142	8.68	**2,408,165**	7.81

The total fair value of stock options vested during the years ended December 31, 2023 and 2022, was$9.2 million and $3.7 million, respectively.

Unvested stock options as of December 31, 2023 and 2022, were as follows:

	Number of Unvested Shares December 31,	
Exercise Price	**2023**	**2022**
$2.848 to $10.00	5,614,977	1,824,285
$30.01 to $50.00	—	17,121
Total number of unvested stock options	**5,614,977**	**1,841,406**

As of December 31, 2023, there was $13.8 million of total unrecognized compensation cost related to unvested share-based compensation arrangements granted under the A&R 2014 Plan and 2023 Plan. That compensation cost is expected to be recognized over a weighted-average period of 2.87 years. There was no stock-based compensation expense related to performance-based awards recognized during the year ended December 31, 2023. There was $0.4 million of stock-based compensation expense related to performance-based awards recognized during the year ended December 31, 2022.

L. Fair Value of Financial Instruments

The accounting standard for fair value measurements provides a framework for measuring fair value and requires disclosures regarding fair value measurements. Fair value is defined as the price that would be received to sell an asset or paid to transfer a liability in an orderly transaction between market participants at the measurement date, based on the Company's principal or, in absence of a principal, most advantageous market for the specific asset or liability.

The Company uses a three-tier fair value hierarchy to classify and disclose all assets and liabilities measured at fair value on a recurring basis, as well as assets and liabilities measured at fair value on a non-recurring basis, in periods subsequent to their initial measurement. The hierarchy requires the Company to use observable inputs when available, and to minimize the use of unobservable inputs, when determining fair value. The three tiers are defined as follows:

- Level 1—Observable inputs that reflect quoted market prices (unadjusted) for identical assets or liabilities in active markets;
- Level 2—Observable inputs other than quoted prices in active markets that are observable either directly or indirectly in the marketplace for identical or similar assets and liabilities; and
- Level 3—Unobservable inputs that are supported by little or no market data, which require the Company to develop its own assumptions.

The carrying amounts of certain financial instruments, including cash and cash equivalents, accounts and other receivables, accounts payable and accrued expenses, the Nantahala Note, and the margin account approximate their respective fair values due to the short-term nature of such instruments.

Assets and Liabilities Measured at Fair Value on a Recurring Basis

The Company evaluates its financial assets and liabilities subject to fair value measurements on a recurring basis to determine the appropriate level in which to classify them for each reporting period. This determination requires significant judgments to be made. The following table summarizes the conclusions reached regarding fair value measurements as of December 31, 2023 and 2022 (in thousands):

	Balance at December 31, 2023	**Quoted Prices in Active Markets for Identical Assets (Level 1)**	**Significant Other Observable Inputs (Level 2)**	**Significant Unobservable Inputs (Level 3)**
CVR liability (Note R)	$ 7,262	$ —	$ —	$ 7,262
Warrant liabilities	$ 16,100	—	—	$ 16,100
Total liabilities	$ 23,362	$ —	$ —	$ 23,362
Securities:				
U.S. Treasury securities	$ 24,688	$ 24,688	$ —	$ —
Total assets	$ 24,688	$ 24,688	$ —	$ —

	Balance at December 31, 2022	**Quoted Prices in Active Markets for Identical Assets (Level 1)**	**Significant Other Observable Inputs (Level 2)**	**Significant Unobservable Inputs (Level 3)**
Warrant liabilities (As Restated)	$ 10,202	$ —	$ —	$ 10,202
Total liabilities	$ 10,202	$ —	$ —	$ 10,202
Securities:				
U.S. government-sponsored agency securities	$ 7,189	$ —	$ 7,189	$ —
U.S. Treasury securities	9,711	9,711	—	—
Total assets	$ 16,900	$ 9,711	$ 7,189	$ —

The common stock warrant liabilities were recorded at fair value using the Black-Scholes option pricing model. The following assumptions were used in determining the fair value of the warrant liabilities valued using the Black-Scholes option pricing model as of December 31, 2023, and 2022:

	December 31, 2023	December 31, 2022
Risk-free interest rate	3.76% - 4.12%	4.01% - 4.12%
Volatility	62.01% - 92.42%	98.33% - 103.01%
Dividend yield	—%	—%
Expected term (years)	2.0 - 4.9	3.0 - 4.0
Weighted average fair value	2.94	2.94

The following table is a reconciliation for the common stock warrant liabilities measured at fair value using Level 3 unobservable inputs (in thousands):

Balance at December 31, 2021 (As Restated)	$	25,032
Change in fair value measurement (As Restated)		(14,830)
Balance at December 31, 2022 (As Restated)	$	10,202
Fair value of warrants issued		4,500
Change in fair value measurement		1,398
Balance at December 31, 2023	$	16,100

For the years ended December 31, 2023, and 2022, the changes in fair value of the warrant liabilities primarily resulted from the volatility of the Company's common stock and the change in the risk-free interest rates.

M. Income Taxes

The Company's financial statements include a total state tax expense of$31,000 related to research and development credits on a loss before income taxes of approximately $26.8 million for the year ended December 31, 2022. The Company did not recognize any state tax expense during the year ended December 31, 2023. The Company met the requirements to receive a tax credit of $0.8 million for losses in Denmark resulting from research and development costs, which is included in interest and other income, net for the year ended December 31, 2022. A reconciliation of the difference between the (expense)/benefit for income taxes and income taxes at the statutory U.S. federal income tax rate is as follows (in thousands, except amounts pertaining to rate which are shown as a percentage):

	Year ended December 31,	
	2023	2022 (As Restated)
Federal statutory rate	21.00%	21.00%
Effect of:		
Change in valuation allowance	(23.29)	(34.77)
Return to provision and deferred true-up	(2.41)	(5.37)
Federal research and development credit	4.09	1.82
State research and development credit	—	0.12
Change in rate	(0.01)	0.80
State tax benefit (net of federal)	5.19	4.78
Warrant liability	—	12.39
Stock-based compensation	(0.20)	(1.09)
Global intangible low-taxed income credit	(3.41)	(1.92)
Foreign research and development excess benefit	(0.86)	2.17
Other	(0.10)	0.19
Federal income tax provision effective rate	—%	0.12%

The components of deferred tax assets and liabilities are as follows (in thousands):

	December 31,	
	2023	2022
Deferred tax assets relating to:		
Net operating loss carryforwards	$ 90,743	$ 66,373
Research and development tax carryforward	20,670	6,900
Stock-based compensation	5,154	4,185
174 expenses	30,241	3,730
Right-of-use liability	265	359
Property and equipment	23	22
Other deferred tax assets	1,951	598
Total gross deferred tax assets	149,047	82,167
Deferred tax liabilities relating to:		
Right-of-use asset	249	(336)
Intangibles	14,473	—
Total gross deferred tax liabilities	14,722	(336)
Deferred tax assets less liabilities	134,324	81,831
Valuation allowance	(134,488)	(81,831)
Net deferred tax asset (liability)	$ (164)	$ —

In assessing the realizability of deferred tax assets, management considers whether it is more likely than not that some portion or all of the deferred tax assets will not be realized. The ultimate realization of deferred tax assets is dependent upon the generation of future taxable income during the periods in which those temporary differences become deductible. Management considers the scheduled reversal of deferred tax liabilities, projected future taxable income and tax planning strategies in making this assessment. Based upon the level of historical taxable income (losses) and projections for future taxable income (losses) over the periods in which the deferred tax assets are deductible, management believes it is more likely than not that material amount of the Company's net DTA will not be realized and as such valuation allowance has been recorded excluding the portion of the indefinite lived DTLs cannot be utilized as future source of income against indefinite lived DTAs.

The Company recorded refundable research and development tax credit as other income and not income tax under ASC 740 in the consolidated statement of operations for the years ended December 31, 2022, and 2023. These refundable tax credits are a result of increased qualified research and development spending in certain jurisdictions which allow for a refundable credit even when the Company has no current period income tax expense.

In accordance with the Tax Cuts and Jobs Act of 2017, the Company evaluated its plans for reinvestment or repatriation of current and future earnings of foreign operations and determined to indefinitely reinvest current and future earnings of foreign operations in the foreign operation. The Company has not repatriated funds to the U.S. to satisfy domestic liquidity needs, nor does the Company anticipate the need to do so. If in the foreseeable future, the Company can no longer demonstrate that these earnings are indefinitely reinvested, a deferred tax liability will be recognized.

The Company has federal operating loss carryforwards totaling $350 million, $145.3 million of which, if not utilized, will begin to expire in 2027 and $204.7 million of which have no expiration date. The Company also has certain state net operating loss carryforwards totaling $340.3 million, which, if not utilized, will begin to expire in 2027. The Company also has Denmark net operating loss carryforwards totaling $4.7 million which has indefinite carryforward period in Denmark. Due to potential ownership changes that may have occurred or would occur in the future, Internal Revenue Code Section 382 may place additional limitations on the Company’s ability to utilize the net operating loss carryforward.

ASC 740-10, Accounting for Uncertainty in Income Taxes, uses the term “more likely than not” to evaluate whether or not a tax position will be sustained upon examination. The Company has not identified any tax positions that do not meet the more likely than not threshold.

N. Net Loss Per Share

For all periods presented herein, the Company did not use the two-class method to compute net loss attributable to common stockholders per share of common stock, even though it had issued securities, other than common stock, that contractually entitled the holders to participate in dividends and earnings, because these holders are not obligated to participate in a loss. The two-class method requires earnings for the period to be allocated between common stock and participating securities based upon their respective rights to receive distributed and undistributed earnings.

Under the two-class method, for periods with net income attributable to common stockholders, basic net income attributable to common stockholders per share of common stock is computed by dividing the undistributed net income attributable to common stockholders by the weighted average number of shares of common stock outstanding during the period. Undistributed net income attributable to common stockholders is computed by subtracting from net income the portion of current period earnings that participating securities would have been entitled to receive pursuant to their dividend rights had all of the period's earnings been distributed and subtracting the actual or deemed dividends declared. No such adjustment to earnings is made during periods with a net loss as the holders of the participating securities have no obligation to fund losses. Diluted net income attributable to common stockholders per share of common stock is computed under the two-class method by using the weighted average number of shares of common stock outstanding plus the potential dilutive effects of stock options and warrants. In addition to analyzing under the two class method, the Company analyzes the potential dilutive effect of stock options and warrants, under the treasury-stock method when calculating diluted income (loss) attributable to common stockholders per share of common stock, in which it is assumed that the stock options and warrants convert into common stock at the beginning of the period or date of issuance, if the stock option or warrant was issued during the period. The Company reports the more dilutive of the approaches (two-class or treasury-stock/if-converted) as its diluted net income (loss) attributable to common stockholders per share of common stock during the period.

As noted above, for all periods presented herein, the Company did not utilize the two-class approach as the Company was in a net loss position and the holders of the participating securities have no obligation to fund losses. The Company did analyze diluted net loss attributable to common stockholders per share of common stock under the treasury-stock/if-converted method and noted that all outstanding stock options and warrants were anti-dilutive for the periods presented. For all periods presented, basic net loss attributable to common stockholders per share of common stock was the same as diluted net loss attributable to common stockholders per share of common stock.

The following securities, presented on a common stock equivalent basis, have been excluded from the calculation of weighted average number of shares of common stock outstanding because their effect is anti-dilutive:

	December 31,	
	2023	**2022**
Awards under equity incentive plans	1,728,885	2,456,407
Common stock warrants	5,603,729	4,252,600
Total securities excluded from the calculation of weighted average number of shares of common stock outstanding	7,332,614	6,709,007

A reconciliation from net loss to basic net loss attributable to common stockholders per share of common stock and diluted net loss attributable to common stockholders per share of common stock for the years ended December 31, 2023 and 2022, is as follows (in thousands):

	December 31,	
	2023	**2022 (As Restated)**
Basic and diluted net loss per share of common stock:		
Net loss, basic and diluted	$ (46,049)	$ (26,772)
Weighted average number of shares of common stock outstanding, basic and diluted	35,452	34,489
Basic and diluted net loss per share of common stock	$ (1.30)	$ (0.78)

O. Leases

The Company has operating and finance leases for office space, laboratory facilities and various laboratory equipment, furniture and office equipment and leasehold improvements. The Company determines if an arrangement is a lease at contract inception. Lease assets and lease liabilities are recognized based on the present value of lease payments over the lease term at the commencement date. The Company does not separate lease and non-lease components. Leases with a term of 12 months or less at commencement are not recorded on the consolidated balance sheets. Lease expense for these arrangements is recognized on a straight-line bases over the lease term. The Company's leases have remaining lease terms of less than 1 year to approximately 4 years, some of which include options to extend the leases for up to 5 years, and some which include options to terminate the leases within 1 year.

Effective June 1, 2021, the Company agreed to sublease office space in Florida, comprised of one of the two contiguous suites, under a non-cancelable operating lease, which expires in February 2026.

The components of lease expense were as follows (in thousands):

	Year Ended December 31,	
Lease Cost	**2023**	**2022**
Finance lease cost:		
Amortization of right-of-use assets	$ 102	$ 129
Interest on lease liabilities	—	2
Total finance lease cost	102	131
Operating lease cost	437	418
Short-term lease cost	171	210
Variable lease cost	40	39
Less: sublease income	(157)	(157)
Total lease costs	$ 593	$ 641

Supplemental cash flow information related to leases was as follows (in thousands):

	Year Ended December 31,	
	2023	**2022**
Cash paid for amounts included in the measurement of lease liabilities:		
Operating cash flows from finance leases	$ —	$ 2
Financing cash flows from finance leases	5	16
Operating cash flows from operating leases	552	522
Operating cash flows from short-term leases	171	210
Operating cash flows from variable lease costs	40	39
Right-of-use assets obtained in exchange for lease liabilities:		
Finance leases	$ —	$ —
Operating leases	—	123

Supplemental balance sheet information related to leases was as follows (in thousands, except weighted average remaining lease term and weighted average discount rate):

	December 31,	
	2023	2022
Finance Leases		
Property and equipment, at cost	$ 1,031	$ 1,031
less: accumulated depreciation and amortization	(882)	(780)
Property and equipment, net	$ 149	$ 251
Other current liabilities	$ —	$ 6
Other long-term liabilities	—	—
Total finance lease liabilities	$ —	$ 6
Operating Leases		
Operating lease right-of-use assets	$ 790	$ 988
Total operating lease right-of-use assets	$ 790	$ 988
Current portion of operating lease liabilities	$ 543	$ 480
Operating lease liabilities, less current portion	456	843
Total operating lease liabilities	$ 999	$ 1,323
Weighted Average Remaining Lease Term		
Finance leases (in years)	0	1
Operating leases (in years)	2	3
Weighted Average Discount Rate		
Finance leases	14.3%	14.3%
Operating leases	7.6%	7.3%

Maturities on lease liabilities were as follows (in thousands):

Year Ending December 31,	Finance Leases	Operating Leases
2024	$ —	$ 644
2025	—	389
2026	—	30
Total lease payments	—	1,063
Less: future interest expense	—	(64)
Lease liabilities	$ —	$ 999

P. Employee Benefit Plan

The Company has a 401(k) retirement plan (the "401(k) Plan") that covers substantially all employees. The Company may provide a discretionary match with a maximum amount of 4% of the participant's compensation, which vests immediately. The Company made matching contributions under the 401(k) Plan of approximately $0.3 million and $0.2 million for the years ended December 31, 2023 and 2022, respectively.

The Company has a discretionary profit-sharing plan (the "Profit Sharing Plan") that covers all employees. Employees become eligible participants in the Profit Sharing Plan once they have provided three years of service to the Company. The Company made no contributions to the Profit Sharing Plan in 2023 or 2022.

Q. Significant Events

On January 6, 2023, the Company's Board of Directors (the "Board") appointed Richard W. Pascoe to serve as the Company's Chief Executive Officer, effective immediately. Concurrently with his appointment as Chief Executive Officer, Mr. Pascoe stepped down as the Company's Executive Chairman. Mr. Pascoe continued to serve as a member of the Board until the date of the Company's 2023 Annual Meeting of Stockholders (the "Annual Meeting"), which was held on April 25, 2023. Mr. Pascoe was designated as the Company's principal executive officer, succeeding Travis C. Mickle, Ph.D., the Company's President and former Chief Executive Officer, in such role. On January 6, 2023, Dr. Mickle resigned from his role (i) as Chief Executive Officer, effective immediately, and (ii) as President and as a member of the Board, in each case, effective as of the date of the Annual Meeting. Additionally, on January 6, 2023, the Board appointed Matthew R. Plooster, a member of the Board, as the Chairman of the Board.

In connection with Mr. Pascoe's appointment as the Company's Chief Executive Officer, the Company and Mr. Pascoe entered into an amendment to the employment agreement, dated November 5, 2021, by and between the Company and Mr. Pascoe (the "Amendment"). Pursuant to the Amendment, Mr. Pascoe became entitled to receive an option under the A&R 2014 Plan to purchase 700,000 shares of the Company's common stock at an exercise price equal to the closing price of the Company's common stock on January 9, 2023. The option will vest in four equal annual installments, with the first such installment occurring on January 6, 2024 (subject to Mr. Pascoe's continued service to the Company through the applicable vesting date).

In connection with the management transition, the Company entered into (i) a transition agreement with Dr. Mickle (the "Mickle Transition Agreement") and (ii) a consulting agreement with Dr. Mickle (the "Consulting Agreement"). Pursuant to the terms of the Mickle Transition Agreement, subject to his timely delivering a release of claims in the Company's favor, Dr. Mickle will receive severance payments and benefits consisting of (i) continued payment of his base salary for 18 months following the date on which Dr. Mickle's employment with the Company ends (the "Separation Date"), (ii) up to 18 months of continued medical, dental and vision coverage pursuant to COBRA and (iii) a one-time, lump sum bonus payment equal to a pro rata amount of his annual performance-based target bonus for the year in which the Separation Date occurs. In addition, immediately prior to the Separation Date, all outstanding options to purchase the Company's common stock held by Dr. Mickle will be vested in full, and such accelerated vested options may be exercised through the later of (i) the 18-month anniversary of the date of the Transition Agreement and (ii) the date of the termination of the Consulting Agreement. Pursuant to the terms of the Consulting Agreement, Dr. Mickle has agreed to provide consulting services until the first anniversary of the Annual Meeting. In exchange for such services, Dr. Mickle will receive consulting fees of $40,000 per month. In addition, Dr. Mickle was granted, under the A&R 2014 Plan, 547,945 performance-based restricted stock units, which will vest in full upon the timely achievement of a clinical and development milestone, subject to forfeiture upon certain disqualifying events. As of December 31, 2023, the Company had accrued severance expense recorded within accounts payable and accrued expenses of approximately $0.6 million in connection with the Mickle Transition Agreement.

At the Annual Meeting, each of John B. Bode, Douglas W. Calder, and Corey Watton was elected as a director of the Company and each of Richard W. Pascoe, Christopher A. Posner, and David S. Tierney ceased serving on the Company's Board of Directors. After the Annual Meeting, the Company's Board of Directors accepted the resignation of Richard W. Pascoe from his role as Chief Executive Officer on May 5, 2023, effective June 1, 2023, and appointed Tamara A. Favorito as the Chair of the Board of Directors. In connection with Mr. Pascoe's resignation, the Company entered into a transition agreement with Mr. Pascoe (the "Pascoe Transition Agreement"). Pursuant to the terms of the Pascoe Transition Agreement, Mr. Pascoe is entitled to receive severance payments and benefits consisting of (i) continued payment of his base salary for 12 months following the date on which Mr. Pascoe's employment with the Company ends (the "Separation Date"), (ii) up to 12 months of continued medical, dental and vision coverage pursuant to COBRA, (iii) an amount equal to Mr. Pascoe's target annual bonus, pro-rated through the Separation Date and (iv) accelerated vesting of his outstanding equity awards. In addition, the exercise period of vested options to purchase the Company's common stock held by Mr. Pascoe will be extended through the nine-month anniversary of the Separation Date. As of December 31, 2023, the Company had accrued severance expense recorded within accounts payable and accrued expenses of approximately $0.3 million in connection with the Pascoe Transition Agreement.

On May 3, 2023, Matthew R. Plooster and Joseph B. Saluri indicated to the Board of Directors that they do not intend to stand for re-election at the Company's 2024 Annual Meeting o Stockholders, and that they intend to step down from the Board of Directors as soon as replacements are found.

In May 2023, the Board of Directors appointed Christal M. M. Mickle, Co-Founder and Chief Development Officer, to serve as interim President and Chief Executive Officer effective on June 1, 2023.

On August 7, 2023, the Board of Directors appointed Thomas D. Anderson as a Class III director, with a term expiring at the Company's annual meeting of stockholders to be held in 2024 or until his earlier death, resignation, or removal.

On August 7, 2023, Mr. Plooster resigned from the Board of Directors effective immediately after Mr. Anderson's appointment.

On October 7, 2023, the Board of Directors appointed Neil F. McFarlane to serve as the Company's President and Chief Executive Officer, effective October 10, 2023. Concurrently with his appointment as President and Chief Executive Officer, Mr. McFarlane was appointed to serve as a member of the Board of Directors. Mr. McFarlane was designated as the Company's principal executive officer, succeeding Christal M.M. Mickle, the Company's former interim President and Chief Executive Officer. Ms. Mickle continues to serve in her role as Chief Development Officer. In connection with Mr. McFarlane's appointment as the Company's President and Chief Executive Officer, the Company and Mr. McFarlane entered into an employment agreement, effective October 10, 2023 (the "Employment Agreement"). Pursuant to the Employment Agreement, Mr. McFarlane is entitled to (i) an annual base salary of $700,000, (ii) an annual performance-based bonus targeted at 60% of his annual base salary, (iii) an option to purchase 600,000 shares of the Company's common stock at an exercise price equal to the closing price of the Company's common stock on October 10, 2023, and (iv) restricted stock units covering 200,000 shares of the Company's common stock. The option and restricted stock units were granted to Mr. McFarlane as inducement awards under the 2023 Plan. The option and restricted stock units will vest in four equal annual installments, with the first such installment occurring on October 10, 2024 (subject to Mr. McFarlane's continued service to the Company through the applicable vesting date). In addition, Mr. McFarlane will receive commuting and relocation assistance in connection with his move to Florida. Upon a termination of Mr. McFarlane's termination without cause by the Company or resignation for good reason, Mr. McFarlane is entitled to receive (i) an amount of cash equal to 1.0 times annual base salary, (ii) a pro-rated target annual bonus for the year in which termination occurs, (iii) twelve months of Company paid COBRA continuation coverage, and (iv) full vesting of his outstanding and unvested equity awards. However, if any such termination occurs within one month prior to or six months after a change in control, he will instead receive (i) an amount of cash equal to 1.5 times annual base salary, (ii) a target annual bonus for the year in which termination occurs, (iii) eighteen months of Company paid COBRA continuation coverage, and (iv) full vesting of his outstanding and unvested equity awards. Upon Mr. McFarlane's termination due to death or disability, Mr. McFarlane will receive a pro-rated target annual bonus. Mr. McFarlane is also subject to 12-month post-termination non-competition and non-solicitation restrictions.

On October 10, 2023, Joseph B. Saluri, a member of the Board of the Company, retired from such position, effective immediately after the appointment of Mr. McFarlane.

In January 2024, the Board of Directors appointed Adrian Quartel, M.D, FFPM, as Chief Medical Officer.

On January 20, 2024, the Board of Directors appointed Alvin Shih, M.D., M.B.A as a Class III director, with a term expiring at the Company's 2024 Annual Meeting of Stockholders or until his earlier death, resignation, or removal.

R. Merger

On August 30, 2023, in connection with the Merger Agreement with Acer, the following transactions occurred prior to Closing:

- *Bridge Loan* - Zevra and Acer entered into a bridge loan agreement (the "Bridge Loan Agreement"), providing for Zevra to make loans (collectively, the "Bridge Loan") to Acer up to an aggregate principal amount of $16.5 million. The Bridge Loan was provided to Acer to support its termination agreement with Relief Therapeutics Holding SA ("Relief") and to provide Acer with working capital, including for payments of accounts payable to support the commercial launch of OLPRUVA and the development of celiprolol pending the Merger's closure. On October 31, 2023, the Company and Acer entered into an amendment to the Bridge Loan Agreement, which increased the aggregate principal amount available under the loan from $16.5 million to $18.0 million.

- *Purchase of Acer's Term Loans* - Zevra purchased certain indebtedness of Acer held by Nantahala Capital Management, LLC ("Nantahala). Under the loan purchase with Nantahala, certain of its affiliates and certain other parties (collectively with Nantahala, "Nantahala Holders") Zevra purchased (i) an original senior secured term loan facility made available to Acer in an aggregate amount of $6.5 million and funded on March 14, 2022, and (ii) an additional senior secured term loan made to Acer in an aggregate amount of $7.0 million in a single borrowing which funded on January 31, 2023 for (1) $12.0 million in cash; (2) 98,683 shares of Zevra Common Stock; and (3) a secured Promissory Note payable by Zevra to Nantahala in the original principal amount $5.0 million. These were recorded as receivables form Acer and were treated as a settlement of a preexisting relationship in connection with the closing of the transaction and recorded as a component of purchase consideration.

- *Purchase of Acer's Convertible* Notes ("Marathon Convertible Notes")- Under the Note Purchase Agreement with the Nantahala Holders, Zevra purchased the Marathon Convertible Notes that Nantahala had acquired on June 16, 2023. Zevra acquired the Marathon Convertible Notes in exchange for the issuance of 2,171,038 shares of Zevra Common Stock at $5.0667 per share for a total purchase price of $11.0 million.

- *Amendment to IP License Agreement and IP Termination Agreement:* As a condition to entering into the Merger Agreement, Acer and Relief Therapeutics Holding AG ("Relief") entered into the Exclusive License Agreement and the Termination Agreement terminating the collaboration and license agreement, dated March 19, 2021, by and between Acer and Relief. Pursuant to the Exclusive License Agreement, Relief holds exclusive development and commercialization rights for OLPRUVA in the European Union, Liechtenstein, San Marino, Vatican City, Norway, Iceland, Principality of Monaco, Andorra, Gibraltar, Switzerland, United Kingdom, Albania, Bosnia, Kosovo, Montenegro, Serbia and North Macedonia (Geographical Europe). Acer has the right to receive a royalty of up to 10.0% of the net sales of OLPRUVA in Geographical Europe. In accordance with the terms of the Termination Agreement, Relief received an upfront payment from Acer of $10.0 million (which payment was funded with the Bridge Loan described above) with an additional payment of $1.5 million due on the first-year anniversary of the $10.0 million payment. Acer also agreed to pay a 10.0% royalty on net sales of OLPRUVA worldwide, excluding Geographical Europe, and 20.0% of any value received by Acer from certain third parties relating to OLPRUVA licensing or divestment rights, all of the foregoing which are capped at $45.0 million, for total payments to Relief of up to $56.5 million.

In connection with the closing of the Merger on November 17, 2023, each share of common stock of Acer was converted into the right to receive (i) 0.1210 fully paid and non-assessable shares of common stock of Zevra, par value $0.0001 per share , and (ii) one non-transferable contingent value right ("CVR") to be issued by Zevra, which will represent the right to receive one or more contingent payments up to an additional $76 million upon the achievement, if any, of certain commercial and regulatory milestones for Acer's OLPRUVA and celiprolol products within specified time periods. Certain additional cash payments are also possible pursuant to the CVRs with respect to milestones involving Acer's early-stage program ACER-2820 (emetine).

The assets acquired and liabilities assumed were recorded based on their acquisition date fair values. Consideration for the Merger was $72.6 million and consists of (i) approximately 2.96 million shares of Zevra common stock valued at $12.8 million, (ii) the Bridge Loan advances of $17.8 million, (iii) $12.0 million in cash paid to Nantahala; (iv) 2.27 million shares of Zevra Common Stock issued to Nantahala valued at $11.5 million based on the VWAP of shares of Zevra Common Stock during the 20 consecutive trading days ending on the trading date prior to August 30, 2023; (v) a secured promissory note payable by Zevra to Nantahala in the original principal amount of $5.0 million, as disclosed in Note C, (vi) $8.5 million in the estimated fair value of contingent consideration related to the CVRs, (vii) approximately 0.9 million shares of Zevra Common Stock issued to a former holder of Acer warrants valued at $4.0 million based on Zevra's common stock price on the Effective Date and (viii) $1.0 million in notes payable paid by the Company on Acer's behalf. In addition, effective as of immediately prior to the Effective Time, all of the outstanding and unexercised Acer stock options were automatically cancelled and ceased to exist without any cash or other consideration being paid or provided in respect thereof. The following purchase price allocation reflects the preliminary estimates of and assumptions related to the fair values of assets acquired and liabilities assumed:

Assets		
Cash	$	575
Prepaid expenses		278
Other current assets		11
Inventory		9,376
Property, plant, and equipment		35
Other noncurrent assets		209
Approved product - OLPRUVA		68,000
IPR&D - celiprolol		2,000
Goodwill acquired		4,538
		85,022
Liabilities		
Accounts payable and accrued expenses	$	10,718
Deferred collaboration funding		1,500
Operating lease liabilities		175
		12,393
Fair Value of Net Assets Acquired	$	72,629

The preliminary fair values assigned to the tangible and intangible assets acquired and liabilities assumed were determined using an income approach based on management's estimates and assumptions, as well as other information compiled by management, including third-party valuations that utilize customary valuation procedures and techniques. These preliminary fair values are subject to change within the one-year measurement period. The estimated fair values were developed by discounting future net cash flows to their present value at market-based rates of return. The goodwill acquired represents the excess of the purchase price and related costs over the value assigned to the net tangible and identifiable intangible assets of the business acquired. The useful lives of the intangible assets for amortization purposes were determined by considering the period of expected cash flows used to measure the fair values of the intangible assets adjusted as appropriate for entity-specific factors including legal, regulatory, contractual, competitive, economic and other factors that may limit the useful life. The marketed product asset is amortized on a straight-line basis over its estimated useful life. As of December 31, 2023, the IPR&D project had not been completed or abandoned and, therefore, the IPR&D intangible asset is not currently subject to amortization.

The results of operations and changes in stockholders' equity for Acer were included in the Company's consolidated financial statements beginning November 18, 2023. Acer had total operating revenue of $42,000 and a net loss of $6.8 million for the period from November 18, 2023, through December 31, 2023.

The following pro forma combined results of operations present the acquisition as if it had occurred on January 1, 2022. The pro forma combined results of operations do not necessarily represent the Company's consolidated results of operations had the acquisition occurred on the date assumed, nor are these results necessarily indicative of the Company's future consolidated results of operations. The Company expects to realize certain benefits from integrating Acer into the Company and to incur certain one-time costs. The pro forma combined results of operations do not reflect these benefits or costs.

	Year Ended December 31, 2023	Year Ended December 31, 2022
Pro forma revenue	$ 27,705	$ 10,458
Pro forma net loss	(78,875)	(80,652)

The Company expensed approximately $2.2 million of acquisition-related costs during the year ended December 31, 2023, which is included in selling, general, and administrative expenses in the consolidated statement of operations.

Cancellation of Acer Warrant

On November 22, 2023, the Company sold an aggregate of 917,934 shares of its common stock to a healthcare focused investment fund (the "Investor") to cancel a warrant held by the Investor to purchase 2,920,306 shares of common stock of Acer. The shares of common stock were offered and sold to the Investor in a registered direct offering without an underwriter or placement agent.

Contingent Consideration

Contingent consideration liabilities relate to our liabilities arising in connection with the CVRs issued as a result of the Merger. The contingent consideration is classified as Level 3 in the fair value hierarchy. The fair value is measured based on a Monte Carlo simulation or a scenario-based method, depending on the earn-out achievement objectives, utilizing projections about future performance. Significant inputs include volatility and projected financial information, including projections representative of a market participant's view of the expected cash payments associated with the agreed upon regulatory milestones based on probabilities of technical success, timing of the potential milestone events for the compounds, and estimated discount rates.

The following table provides a reconciliation of the beginning and ending balances related to the contingent consideration liabilities for the CVRs (dollars in thousands):

Balance at December 31, 2022	$ 0
Initial estimate of contingent consideration at the Effective Time	8,562
Change in fair value recognized in earnings	(1,300)
Balance at December 31, 2023	$ 7,262

For the year ended December 31, 2023, the Company recorded a $1.3 million gain on the change in fair value of contingent consideration, primarily due to changes in market data and revenue projections.

S. Subsequent Events

The Company evaluated events and transactions occurring subsequent to December 31, 2023, through April, 2024, the date the accompanying financial statements were issued. During this period, there were no subsequent events that required recognition in the accompanying consolidated financial statements nor were there any additional nonrecognized subsequent events that required disclosure.

EXHIBITS

Exhibit No.	Description
2.1***	Agreement and Plan of Merger dated as of August 30, 2023, by and among Zevra Therapeutics, Inc., Aspen Z Merger Sub, Inc., and Acer Therapeutics Inc. (incorporated herein by reference to the Registrant's Current Report on Form 8-K as filed with the SEC on August 31, 2023).
3.1	Amended and Restated Certificate of Incorporation of Zevra Therapeutics, Inc.
3.1.1	Certificate of Amendment of Amended and Restated Certificate of Incorporation of the Company, effective as of December 23, 2020 (incorporated herein by reference to Registrant's Current Report on Form 8-K as filed with the SEC on December 23, 2020).
3.1.2	Certificate of Elimination of Series A Convertible Preferred Stock of Zevra Therapeutics, Inc. (incorporated herein by reference to the Registrant's Current Report on Form 8-K as filed with the SEC on June 23, 2021).
3.1.3	Certificate of Elimination of Series B-1 Convertible Preferred Stock of Zevra Therapeutics, Inc. (incorporated herein by reference to the Registrant's Current Report on Form 8-K as filed with the SEC on June 23, 2021).
3.1.4	Certificate of Elimination of Series B-2 Convertible Preferred Stock of Zevra Therapeutics, Inc. (incorporated herein by reference to the Registrant's Current Report on Form 8-K as filed with the SEC on June 23, 2021).
3.1.5	Certificate of Amendment of Amended and Restated Certificate of Incorporation of Zevra Therapeutics, Inc. (incorporated herein by reference to the Registrant's Current Report on Form 8-K as filed with the SEC on February 24, 2023).
3.2	Amended and Restated Bylaws, as currently in effect, of Zevra Therapeutics, Inc. (incorporated herein by reference to the Registrant's Current Report on Form 8-K as filed with the SEC on February 28, 2024).
4.1	Specimen stock certificate evidencing shares of Common Stock (incorporated herein by reference to the Registrant's Annual Report on Form 10-K as filed with the SEC on March 12, 2021).
4.2	Form of Common Stock Purchase Warrant and schedule of holders (incorporated herein by reference to the Registrant's Current Report on Form 8-K as filed with the SEC on January 13, 2021).
4.3	Form of Inducement Warrant (incorporated herein by reference to the Registrant's Current Report on Form 8-K as filed with the SEC on January 26, 2021).
4.4	Form of Inducement Warrant (incorporated herein by reference to the Registrant's Current Report on Form 8-K as filed with the SEC on June 23, 2021).
4.5*	Description of the Registrant's Securities Registered Pursuant to Section 12 of the Securities Exchange Act of 1934.
4.6	Form of Common Stock Purchase Warrant (incorporated herein by reference to the Registrant's Current Report on Form 8-K as filed with the SEC on November 20, 2023).
10.1+	Material Supply Agreement, by and between the Registrant and Johnson Matthey, Inc., dated as of November 2, 2009 (incorporated by reference Registrant's Amendment No. 1 to Registration Statement on Form S-1/A (File No. 333-202660) as filed with the SEC on April 3, 2015).
10.2	Payoff letter, dated as of February 8, 2021, by and among the Company and the lenders named therein (incorporated herein by reference to the Registrant's Current Report on Form 8-K as filed with the SEC on February 9, 2021).
10.3	Second Amendment to Senior Secured Convertible Note and Warrant, by and between Registrant and Deerfield Private Design Fund III, L.P., dated January 6, 2016 (incorporated by reference to the Registrant's Current Report on Form 8-K as filed with the SEC on January 11, 2016).
10.3.1	Fourth Amendment to Senior Secured Convertible Note and Warrant, effective as of October 3, 2016, by and between the Registrant and Deerfield Private Design Fund III, L.P. (incorporated herein by reference to the Registrant's Current Report on Form 8-K as filed with the SEC on October 3, 2016).
10.3.2	Amendment to Convertible Note and Warrant Agreement, dated November 20, 2018, between the Company and Deerfield Private Design Fund III, L.P. (as incorporated herein by reference to the Company's Current Report on Form 8-K filed with the SEC on November 20, 2018).
10.3.3	Seventh Amendment to Senior Secured Convertible Note and Sixth Amendment to Warrant, dated February 28, 2019, between the Company and Deerfield Private Design Fund III, L.P. (incorporated herein by reference to the Registrant's Annual Report on Form 10-K as filed with the SEC on March 1, 2019).
10.3.4	Amendment to Senior Secured Convertible Notes and Amendment to Warrant, dated as of February 17, 2020, by and among Registrant and the noteholders party thereto (incorporated herein by reference to the Registrant's Current Report on Form 8-K as filed with the SEC on February 18, 2020)
10.3.5	Amendment to Senior Secured Convertible Notes and Amendment to Warrant, dated as of January 12, 2021, by and among the Company, Deerfield Private Design Fund III, L.P. and Deerfield Special Situations Fund, L.P.(incorporated herein by reference to the Registrant's Current Report on Form 8-K as filed with the SEC on January 13, 2021)
10.4	Warrant to Purchase Shares of Series D Preferred Stock issued to Deerfield Private Design Fund III, L.P., dated as of June 2, 2014 (incorporated herein by reference to the Registrant's Registration Statement on Form S-1 (File No. 333-202660) as filed with the SEC on March 11, 2015).
10.4.1	Form of Stock Purchase Warrant to purchase shares of Series D Convertible Preferred Stock issued in bridge financing, along with a schedule of warrant holders (incorporated herein by reference to the Registrant's Registration Statement on Form S-1 (File No. 333-202660) as filed with the SEC on March 11, 2015).

EXHIBITS, CONTINUED

Exhibit No.	Description
10.5	Amended and Restated Investors' Rights Agreement, dated as of February 19, 2015, by and among the Registrant and certain of its stockholders (incorporated herein by reference to the Registrant's Registration Statement on Form S-1 (File No. 333-202660) as filed with the SEC on March 11, 2015).
10.6+	Agreement to Terminate CLA, by and between MonoSol Rx, LLC and the Registrant, dated as of March 20, 2012 (incorporated herein by reference to the Registrant's Amendment No. 1 to Registration Statement on Form S-1/A (File No. 333-202660) as filed with the SEC on April 3, 2015).
10.7#	Incentive Stock Plan, as amended to date (incorporated herein by reference to the Registrant's Registration Statement on Form S-1 (File No. 333-202660) as filed with the SEC on March 11, 2015).
10.7.1#	Form of Incentive Stock Option Agreement under Incentive Stock Plan (incorporated herein by reference to the Registrant's Registration Statement on Form S-1 (File No. 333-202660) as filed with the SEC on March 11, 2015).
10.7.2#	Form of Nonqualified Stock Option Agreement under Incentive Stock Plan (incorporated herein by reference to the Registrant's Registration Statement on Form S-1 (File No. 333-202660) as filed with the SEC on March 11, 2015).
10.7.3#	Amended and Restated 2014 Equity Incentive Plan (incorporated herein by reference to the Registrant's Quarterly Report on Form 10-Q as filed with the SEC on August 13, 2021).
10.7.4#	Form of Stock Option Grant Notice and Stock Option Agreement under 2014 Equity Incentive Plan (incorporated herein by reference to the Registrant's Registration Statement on Form S-1 File No. 333-202660) as filed with the SEC on March 11, 2015).
10.7.5#	Form of Restricted Stock Unit Grant Notice and Restricted Stock Unit Agreement under 2014 Equity Incentive Plan (incorporated herein by reference to the Registrant's Registration Statement on Form S-1 File No. 333-202660) as filed with the SEC on March 11, 2015).
10.7.6#	Form of Restricted Stock Award Grant Notice and Restricted Stock Award Agreement under 2014 Equity Incentive Plan (incorporated herein by reference to the Registrant's Quarterly Report on Form 10-Q as filed with the SEC on May 14, 2019)
10.7.7#	2021 Employee Stock Purchase Plan (incorporated herein by reference to the Registrant's Quarterly Report on Form 10-Q as filed with the SEC on August 13, 2021).
10.7.8*	2023 Employment Inducement Award Plan and forms of award agreements thereunder.
10.8#	Eighth Amended and Restated Non-Employee Director Compensation Policy effective February 15, 2023 (incorporated herein by reference to the Registrant's Annual Report on Form 10-K as filed with the SEC on March 7, 2023.
10.9#	Form of Indemnification Agreement with the Registrant's directors and executive officers (incorporated herein by reference to the Registrant's Registration Statement on Form S-1 (File No. 333-202660) as filed with the SEC on March 11, 2015).
10.10#	Amended and Restated Employment Agreement by and between the Registrant and R. LaDuane Clifton, dated as of June 25, 2015 (incorporated herein by reference to the Registrant's Quarterly Report on Form 10-Q as filed with the SEC on August 14, 2015).
10.10.1#	Amendment to Amended and Restated Employment Agreement by and between the Registrant and R. LaDuane Clifton, dated as of October 13, 2015 (incorporated herein by reference to the Registrant's Quarterly Report on Form 10-Q as filed with the SEC on November 13, 2015).
10.11#	Employment Agreement by and between the Registrant and Christal M.M. Mickle, dated as of May 30, 2014 (incorporated herein by reference to the Registrant's Registration Statement on Form S-1 (File No. 333-202660) as filed with the SEC on March 11, 2015).
10.11.1#	Amendment to Employment Agreement by and between the Registrant and Christal M.M. Mickle, dated as of October 13, 2015 (incorporated herein by reference to Exhibit 10.1 to the Registrant's Quarterly Report on Form 10-Q filed with the SEC on November 13, 2015).
10.11.2#	Agreement Regarding Employment Terms, dated as of May 13, 2023, between the Registrant and Christal M.M. Mickle (incorporated herein by reference to the Registrant's Current Report on Form 8-K as filed with the SEC on May 15, 2023).
10.12#	Employment Agreement by and between the Registrant and Travis C. Mickle, Ph.D., dated as of May 30, 2014 (incorporated herein by reference to the Registrant's Registration Statement on Form S-1 (File No. 333-202660) as filed with the SEC on March 11, 2015).
10.12.1#	Amendment to Employment Agreement by and between the Registrant and Travis C. Mickle, Ph.D., dated as of October 13, 2015 (incorporated herein by reference to the Registrant's Quarterly Report on Form 10-Q filed with the SEC on November 13, 2015).
10.12.2#	Transition Agreement by and between the Registrant and Travis C. Mickle, Ph.D., dated as of January 4, 2023, as amended (incorporated herein by reference to the Registrant's Current Report on Form 8-K as filed with the SEC on January 9, 2023).
10.12.3#+	Consulting Agreement by and between the Registrant and Travis C. Mickle, Ph.D., dated as of January 6, 2023 (incorporated herein by reference to the Registrant's Current Report on Form 8-K as filed with the SEC on January 9, 2023).
10.13#	Amended and Restated Employment Agreement by and between the Registrant and Sven Guenther, dated as of April 13, 2016 (incorporated herein by reference to the Registrant's Quarterly Report on Form 10-Q as filed with the SEC on May 13, 2016).
10.14#	Executive Employment Agreement by and between the Registrant and Richard W. Pascoe, dated as of November 5, 2021 (incorporated herein by reference to the Registrant's Current Report on Form 8-K as filed with the SEC on November 10, 2021).
10.14.1#	Amendment to Executive Employment Agreement by and between the Registrant and Richard W. Pascoe, dated as of January 6, 2023 (incorporated herein by reference to the Registrant's Current Report on Form 8-K as filed with the SEC on January 9, 2023).
10.14.2#	Transition Agreement dated as of May 6, 2023, between the Registrant and Richard W. Pascoe (incorporated herein by reference to the Registrant's Current Report on Form 8-K as filed with the SEC on May 8, 2023).

EXHIBITS, CONTINUED

Exhibit No.	Description
10.15	Lease Agreement, by and between the Registrant and BRE/COH FL LLC, dated as of November 3, 2014 (incorporated herein by reference to the Registrant's Annual Report on Form 10-K as filed with the SEC on March 10, 2017).
10.15.1	First Amendment to the Lease Agreement, by and between the Registrant and BRE/COH FL LLC, dated as of April 21, 2015 (incorporated herein by reference to the Registrant's Annual Report on Form 10-K as filed with the SEC on March 10, 2017).
10.15.2	Second Amendment to the Lease Agreement, by and between the Registrant and BRE/COH FL LLC, dated as of December 22, 2015 (incorporated herein by reference to the Registrant's Annual Report on Form 10-K as filed with the SEC on March 10, 2017).
10.15.3	Third Amendment to the Lease Agreement, by and between the Registrant and BRE/COH FL LLC, dated as of July 15, 2016 (incorporated herein by reference to the Registrant's Annual Report on Form 10-K as filed with the SEC on March 10, 2017).
10.17	Purchase Agreement, dated February 17, 2020, by and between the Registrant and Lincoln Park Capital Fund, LLC. (incorporated herein by reference to the Registrant's Current Report on Form 8-K as filed with the SEC on February 18, 2020).
10.18+	Collaboration and License Agreement, dated as of September 3, 2019, by and between the Registrant and Boston Pharmaceuticals Holdings SA (incorporated herein by reference to the Registrant's Current Report on Form 8-K as filed with the SEC on September 4, 2019).
10.19+	Amendment No. 1 to Collaboration and License Agreement, effective as of April 8, 2021, by and between the Company and Commave Therapeutics SA (formerly known as Boston Pharmaceuticals Holdings SA) (incorporated herein by reference to the Registrant's Quarterly Report on Form 10-Q as filed with the SEC on August 13, 2021).
10.20	Form of Inducement Letter (incorporated herein by reference to the Registrant's Current Report on Form 8-K as filed with the SEC on January 26, 2021).
10.21	Form of Inducement Letter (incorporated herein by reference to the Registrant's Current Report on Form 8-K as filed with the SEC on June 23, 2021).
10.22	Equity Distribution Agreement, dated July 2, 2021, by and among the Company, JMP Securities LLC and RBC Capital Markets, LLC (incorporated herein by reference to the Registrant's Current Report on Form 8-K as filed with the SEC on July 2, 2021).
10.23†	Asset Purchase Agreement by and among the Registrant, Zevra Denmark A/S and Orphazyme A/S, in restructuring, dated May 15, 2022 (incorporated herein by reference to the Registrant's Current Report on Form 8-K as filed with the SEC on May 16, 2022).
10.24	Revolving Loan Agreement dated May 31, 2022, by and among the Registrant and Ameris Bank, as lender (incorporated herein by reference to the Registrant's Current Report on Form 8-K as filed with the SEC on June 1, 2022).
10.25	Bridge Loan Agreement dated as of August 30, 2023, by and between the Registrant and Acer Therapeutics Inc. (incorporated herein by reference to the Registrant's Current Report on Form 8-K as filed with the SEC on August 31, 2023).
10.26***	Loan Purchase Agreement dated as of August 30, 2023, by and among the Registrant and Nantahala Capital Management, LLC and the other sellers party thereto (incorporated herein by reference to the Registrant's Current Report on Form 8-K as filed with the SEC on August 31, 2023).
10.27***	Note Purchase Agreement dated as of August 30, 2023, by and among the Registrant and Nantahala Capital Management, LLC and the other sellers party thereto (incorporated herein by reference to the Registrant's Current Report on Form 8-K as filed with the SEC on August 31, 2023).
10.28	Registration Rights Agreement dated as of August 30, 2023, by and among the Registrant and each of the sellers party thereto (incorporated herein by reference to the Registrant's Current Report on Form 8-K as filed with the SEC on August 31, 2023).
10.29	Ninth Amended and Restated Non-Employee Director Compensation Policy (incorporated herein by reference to the Registrant's Quarterly Report on Form 10-Q as filed with the SEC on August 14, 2023.
10.30	Employment Agreement, effective as of October 10, 2023, between the Registrant and Neil F. McFarlane (incorporated herein by reference to the Registrant's Current Report on 8-K as filed with the SEC on October 10, 2023).
10.31*	First Amendment to the Bridge Loan Agreement by and between the Registrant and Acer Therapeutics Inc.
10.32	Contingent Value Rights Agreement, dated as of November 17, 2023, by and among Zevra Therapeutics, Inc., Computershare, Inc., and Computershare Trust Company, N.A. (incorporated by reference to the Registrant's Current Report on 8-K as filed with the SEC on November 20, 2023).
21.1*	Subsidiaries of the Company
23.1*	Consent of Ernst & Young LLP, Independent Registered Public Accounting Firm.
24.1*	Power of Attorney (included on signature page).
31.1*	Certification of the Principal Executive Officer pursuant to Rule 13a-14(a) or Rule 15d-14(a) of the Securities Exchange Act of 1934, as amended.
31.2*	Certification of the Principal Financial Officer pursuant to Rule 13a-14(a) or Rule 15d-14(a) of the Securities Exchange Act of 1934, as amended.
32.1**	Certification of the Principal Executive Officer pursuant to Rule 13a-14(b) of the Securities Exchange Act of 1934, as amended, and 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.
32.2**	Certification of the Principal Financial Officer pursuant to Rule 13a-14(b) of the Securities Exchange Act of 1934, as amended, and 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002.
97.1*, #	Zevra Therapeutics, Inc. Policy for Recovery of Erroneously Awarded Compensation, Effective October 2, 2023
99.1	Securities Purchase Agreement, dated as of November 20, 2023, by and between the Registrant and Armistice Capital Master Fund Ltd. (incorporated herein by reference to the Registrant's Current Report on 8-K as filed with the SEC on November 20, 2023.

EXHIBITS, CONTINUED

Exhibit No.	Description
101.INS*	Inline XBRL Instance Document.
101.SCH*	Inline XBRL Taxonomy Extension Schema Document.
101.CAL*	Inline XBRL Taxonomy Extension Calculation Linkbase Document.
101.DEF*	Inline XBRL Taxonomy Extension Definition Linkbase Document.
101.LAB*	Inline XBRL Taxonomy Extension Label Linkbase Document.
101.PRE*	Inline XBRL Taxonomy Extension Presentation Linkbase Document.
104	Cover Page Interactive Data File (embedded within the Inline XBRL and contained in Exhibit 101)

*	Filed herewith
**	Furnished herewith
***	Pursuant to Item 601(a)(5) of Regulation S-K, schedules and similar attachments have been omitted. The registrant hereby agrees to furnish a copy of any omitted schedule or similar attachment to the SEC upon request.
#	Indicates management contract or compensatory plan.
+	Certain portions of the exhibit, identified by the mark, "[*]", have been omitted because such portions contained information that is both (i) not material and (ii) the type that the Registrant treats as private or confidential.
†	Schedules have been omitted pursuant to Item 601(a)(5) of Regulation S-K. A copy of any omitted schedule will be furnished to the Securities and Exchange Commission upon request; provided, however, that the parties may request confidential treatment pursuant to Rule 24b-2 of the Exchange Act for any document so furnished.

ITEM 16. FORM 10-K SUMMARY

None.

SIGNATURES

Pursuant to the requirements of Section 13 or 15(d) of the Securities Exchange Act of 1934, the registrant has duly caused this report to be signed on its behalf by the undersigned thereunto duly authorized.

Zevra Therapeutics, Inc.

Dated: April 1, 2024

By: /s/ Neil F. McFarlane
Neil F. McFarlane
President and Chief Executive Officer
(Principal Executive Officer)

Dated: April 1, 2024

By: /s/ R. LaDuane Clifton
R. LaDuane Clifton, MBA, CPA
Chief Financial Officer, Secretary and Treasurer
(Principal Financial Officer)

POWER OF ATTORNEY

KNOW ALL BY THESE PRESENTS, that each person whose signature appears below hereby constitute and appoint Neil F. McFarlane and R. LaDuane Clifton, and each of them (with full power to each of them to act alone), as his true and lawful attorneys-in-fact and agents, with full power of substitution and resubstitution in each of them for him and in his name, place and stead, and in any and all capacities, to sign any and all amendments to this report, and to file the same, with all exhibits thereto and other documents in connection therewith, with the Securities and Exchange Commission, granting unto said attorneys-in-fact and agents, and each of them, full power and authority to do and perform each and every act and thing requisite or necessary to be done in and about the premises, as fully to all intents and purposes as he might or could do in person, hereby ratifying and confirming all that said attorneys-in-fact and agents or any of them, or their or his substitute or substitutes, may lawfully do or cause to be done by virtue hereof.

Pursuant to the requirements of the Securities Exchange Act of 1934, this report has been signed below by the following persons on behalf of the registrant and in the capacities and on the dates indicated.

Signature	Title	Date
/s/ Neil F. McFarlane Neil F. McFarlane	President and Chief Executive Officer (Principal Executive Officer)	April 1, 2024
/s/ R. LaDuane Clifton R. LaDuane Clifton, MBA, CPA	Chief Financial Officer, Secretary and Treasurer (Principal Financial Officer)	April 1, 2024
/s/ Timothy J. Sangiovanni Timothy J. Sangiovanni, CPA	Senior Vice President, Corporate Controller (Principal Accounting Officer)	April 1, 2024
/s/ Thomas D. Anderson Thomas D. Anderson	Director	April 1, 2024
/s/ John B. Bode John B. Bode	Director	April 1, 2024
/s/ Douglas W. Calder Douglas W. Calder	Director	April 1, 2024
/s/Wendy Dixon Wendy Dixon	Director	April 1, 2024
/s/ Tamara A. Favorito Tamara A. Favorito	Director	April 1, 2024
/s/ Alvin Shih Alvin Shih	Director	April 1, 2024
/s/ Corey Watton Corey Watton	Director	April 1, 2024

DESCRIPTION OF THE REGISTRANT'S SECURITIES REGISTERED PURSUANT TO SECTION 12 OF THE SECURITIES EXCHANGE ACT OF 1934

Zevra Therapeutics, Inc. ("we," "our," or "us") has one class of securities registered under Section 12 of the Securities Exchange Act of 1934, as amended, our common stock, par value $0.0001 per share, or common stock. The following description of our capital stock is a summary and does not purport to be complete. It is qualified in its entirety by, and should be read in conjunction with, our amended and restated certificate of incorporation, amended and restated bylaws and applicable Delaware law.

General

Under our amended and restated certificate of incorporation we are authorized to issue up to 250,000,000 shares of common stock and 10,000,000 shares of preferred stock, $0.0001 par value per share, all of which are currently undesignated. Our board of directors may establish the rights and preferences of the undesignated preferred stock from time to time.

Common Stock

Voting Rights

Each holder of our common stock is entitled to one vote for each share on all matters submitted to a vote of the stockholders, including the election of directors. Under our amended and restated certificate of incorporation and amended and restated bylaws, our stockholders do not have cumulative voting rights. The affirmative vote of the majority (plurality, in the case of the election of directors) of shares present and entitled to vote generally on a subject matter shall be the act of the stockholders.

Dividends

Subject to preferences that may be applicable to any then-outstanding preferred stock, holders of our common stock are entitled to receive ratably those dividends, if any, as may be declared from time to time by the board of directors out of legally available funds.

Liquidation

In the event of our liquidation, dissolution or winding up, holders of our common stock will be entitled to share ratably in the net assets legally available for distribution to stockholders after the payment of all of our debts and other liabilities and the satisfaction of any liquidation preference granted to the holders of any then-outstanding shares of preferred stock.

Rights and Preferences

Holders of our common stock have no preemptive, conversion or subscription rights and there are no redemption or sinking fund provisions applicable to our common stock. The rights, preferences and privileges of the holders of our common stock are subject to, and may be adversely affected by, the rights of the holders of shares of any series of our preferred stock that we may designate in the future.

Preferred Stock

Pursuant to our amended and restated certificate of incorporation, our board of directors has the authority, without further action by the stockholders (unless such stockholder action is required by applicable law or stock exchange listing rules), to designate and issue up to 10,000,000 shares of preferred stock in one or more series, to establish from time to time the number of shares to be included in each such series, to fix the designations, powers, preferences, privileges and relative participating, optional or special rights and the qualifications, limitations or restrictions thereof, including dividend rights, conversion rights, voting rights, terms of redemption and liquidation preferences, any or all of which may be greater than the rights of our common stock, and to increase or decrease the number of shares of any such series, but not below the number of shares of such series then outstanding. Our board of directors, without stockholder approval, can issue preferred stock with voting, conversion or other rights that could adversely affect the voting power and other rights of the holders of our common stock. Preferred stock could be issued quickly with terms designed to delay or prevent a change in control of our company or make removal of management more difficult. Additionally, the issuance of preferred stock may have the effect of decreasing the market price of our common stock and may adversely affect the voting power of holders of our common stock and reduce the likelihood that our common stockholders will receive dividend payments and payments upon liquidation.

Our board of directors will fix the designations, voting powers, preferences and rights, as well as the qualifications, limitations or restrictions, of the preferred stock of each series that we offer in the certificate of designation relating to that series.

The Delaware General Corporation Law, the law governing corporations in the state of our incorporation, provides that the holders of preferred stock will have the right to vote separately as a class (or, in some cases, as a series) on an amendment to our certificate of incorporation if the amendment would change the par value or, unless the certificate of incorporation provided otherwise, the number of authorized shares of the class or change the powers, preferences or special rights of the class or series so as to adversely affect the class or series, as the case may be. This right is in addition to any voting rights that may be provided for in the applicable certificate of designation.

Outstanding Warrants

Our warrants include a net exercise provision and contain provisions for the adjustment of the exercise price and the number of shares issuable upon the exercise of each warrant in the event of certain stock dividends, stock splits, reorganizations, reclassifications and consolidations. We have also granted registration rights to OTA LLC, or OTA, as more fully described below under "—Registration Rights."

In June 2014, in connection with our entering into a facility agreement, we issued to Deerfield Private Design Fund III, L.P., or Deerfield, a warrant, or the Deerfield Warrant, to purchase 14,423,076 shares of Series D redeemable convertible preferred stock at an exercise price of $0.78 per share, which is exercisable until June 2, 2024. Upon completion of our initial public offering, the Deerfield Warrant automatically converted into a warrant to purchase 120,192 shares of our common stock at an exercise price of $93.60 per share. According to the terms of the Deerfield Warrant, in no event may Deerfield exercise this warrant if such exercise would result in Deerfield beneficially owning more than 4.985% of the then issued and outstanding shares of our common stock. This exercise limitation may not be waived and any purported exercise that is inconsistent with this exercise limitation is null and void. This exercise limitation will not apply to any exercise made immediately prior to a change of control transaction. If Deerfield is only able to exercise the Deerfield Warrant for a limited number of shares due to this exercise limitation, the Deerfield Warrant could subsequently become exercisable to purchase the remainder of the shares as a result of a variety of events. This could occur, for example, if we issue more shares or Deerfield sells some of its existing shares. The Deerfield Warrant includes a net exercise provision and contains provisions for the adjustment of the exercise price and the number of shares issuable upon the exercise of the warrant in the event of certain stock dividends, stock splits, recapitalizations, reclassifications and consolidations. Under the Deerfield Warrant, Deerfield also has the right to demand upon the occurrence of specified events, including a merger, asset sale or other change of control transaction, that we redeem the Deerfield Warrant for a cash amount equal to the Black-Scholes value of the portion of the Deerfield Warrant to be redeemed. If Deerfield chooses not to redeem the Deerfield Warrant upon the occurrence of such an event, we may not enter into any such transaction unless our successor entity assumes in writing all our obligations under both the Deerfield Warrant and the Deerfield facility and provides Deerfield with certain registration rights.

The Deerfield Warrant includes certain exercise price protection provisions pursuant to which the exercise price of the Deerfield Warrant will be adjusted downward on a broad-based weighted average basis if we issue or sell any shares of common stock, convertible securities, warrants or options, at a sale or exercise price per share less than the greater of the Deerfield Warrant's exercise price or the closing sale price of our common stock on our principal market or exchange on the last trading date immediately prior to such issuance or, in the case of a firm commitment underwritten offering, on the date of execution of the underwriting agreement between us and the underwriters for such offering. The sale price, for purposes of this adjustment is measured after giving effect to any underwriting discounts and commissions. This exercise price adjustment does not apply to certain specified sales and in any offering deemed by the Securities and Exchange Commission, or the SEC, to constitute an "at the market offering" as defined in Rule 415 of the Securities Act of 1933, or the Securities Act, of our common stock, the exercise price of the Deerfield Warrant will be adjusted downward pursuant to this anti-dilution adjustment only if such sales are made at a price less than $93.60 per share. In 2021, we entered into two separate warrant inducement transactions which triggered these exercise price protection provision and the exercise price of the Deerfield Warrant was reduced from $93.60 per share to $46.25 per share and finally to $38.34 per share. In December 2022, Deerfield assigned the Deerfield Warrant to OTA, herein referred to as the OTA Warrant.

In January 2021, pursuant to the terms of the underwriting agreement and the December 2020 exchange agreement, we issued warrants to purchase 12,078,361 shares of our common stock, or collectively, the Offering Warrants, in a public offering and in connection with the transactions contemplated under the December 2020 exchange agreement. The Offering Warrants were immediately exercisable and expire on the fifth anniversary of their issuance date, at an exercise price per share of common stock equal to $6.50 per share. The Offering Warrants are exercisable, at the option of each holder, in whole or in part, by delivering to us a duly executed exercise notice, provided that payment in full for the number of shares of our common stock purchased upon such exercise is delivered to us in accordance with the terms of the Offering Warrants. In lieu of making the cash payment otherwise contemplated to be made to us upon such exercise in payment of the aggregate exercise price, the holder may elect instead to receive upon such exercise (either in whole or in part) the net number of shares of common stock determined according to a formula set forth in the Offering Warrants. A holder (together with its affiliates) may not exercise any portion of the Offering Warrant to the extent that the holder and its affiliates and any other person or entities with which such holder would constitute a Section 13(d) "group" would own more than 4.99% of our outstanding common stock immediately after exercise. Except as otherwise provided in the Offering Warrants or by virtue of such holder's ownership of shares of our common stock, the holders of the Offering Warrants do not have the rights or privileges of holders of common stock with respect to the shares of common stock underlying the Offering Warrants, including any voting rights, until they exercise their Offering Warrants. The Offering Warrants provide that holders have the right to participate in distributions or dividends paid on our common stock. In the event of a fundamental transaction, as described in the Offering Warrants and generally including any reorganization, recapitalization or reclassification of our common stock, the sale, transfer or other disposition of all or substantially all of our properties or assets, our consolidation or merger with or into another person, the acquisition of more than 50% of our outstanding common stock, or any person or group becoming the beneficial owner of 50% of the voting power represented by our outstanding common stock, the holders of the Offering Warrants will be entitled to receive upon exercise of the warrants the kind and amount of securities, cash or other property that the holders would have received had they exercised the Offering Warrants immediately prior to such fundamental transaction. In addition, in the event of a fundamental transaction which is approved by our board of directors, the holders of the warrants have the right to require us or a successor entity to redeem the Offering Warrants for cash in the amount of the Black Scholes value of the unexercised portion of the Offering Warrants on the date of the consummation of the fundamental transaction. In the event of a fundamental transaction which is not approved by our board of directors, the holders of the Offering Warrants have the right to require us or a successor entity to redeem the Offering Warrants in the amount of the Black Scholes value of the unexercised portion of the Offering Warrants on the date of the consummation of the fundamental transaction payable in the form of consideration paid to the holders of common stock in such fundamental transaction. The Offering Warrants meet the equity classification requirements and thus are recorded in additional paid-in capital on the balance sheets.

In January 2021, pursuant to the terms of an underwriting agreement, we issued to the underwriter a warrant to purchase 806,932 shares of our common stock, or the Underwriter Warrant. The Underwriter Warrant is subject to substantially the same terms and conditions as the Offering Warrants, provided that the exercise price for the Underwriter Warrant is $8.125 per share. In connection with the closing of the underwriter's partial exercise of its over-allotment option, in February 2021, we issued to the underwriter additional warrants to purchase 18,702 shares of common stock under the same terms as the Underwriter Warrant, collectively these are known as the Underwriter Warrants.

In January 2021, we entered into warrant exercise inducement offer letters, or the January 2021 Inducement Transaction, with certain holders of the Offering Warrants pursuant to which such holders agreed to exercise for cash their existing warrants to purchase 6,620,358 shares of our common stock in exchange for our agreement to issue new warrants, or the January 2021 Inducement Warrants, on substantially the same terms as the existing warrants, except as set forth in the following sentence, to purchase up to 7,944,430 shares of our common stock, which is equal to 120% of the number of shares of our common stock issued upon exercise of the existing warrants. The purchase price of the January 2021 Inducement Warrants was $0.125 per share underlying each January 2021 Inducement Warrant, and the January 2021 Inducement Warrants have an exercise price of $6.36 per share.

In June 2021, we entered into warrant exercise inducement offer letters, or the June 2021 Inducement Transaction, with certain holders of the January 2021 Inducement Warrants pursuant to which such holders agreed to exercise for cash their January 2021 Inducement Warrants to purchase 6,117,509 shares of our common stock in exchange for our agreement to issue new warrants, the June 2021 Inducement Warrants, on substantially the same terms as the January 2021 Inducement Warrants, except as set forth in the following sentence, to purchase up to 1,529,379 shares of our common stock, which is equal to 25% of the number of shares of our common stock issued upon exercise of the January 2021 Inducement Warrants. The purchase price of the June 2021 Inducement Warrants was $0.125 per share underlying each June 2021 Inducement Warrant, and the June 2021 Inducement Warrants have an exercise price of $16.50 per share.

On November 22, 2023, we sold an aggregate of 1,382,489 shares of our common stock and accompanying warrants to purchase up to 1,382,489 shares of our common stock at a price of $4.34 per share to a healthcare focused investment fund (the "Investor") for gross proceeds of approximately $6.0 million and an aggregate of 917,934 shares of our common stock to cancel a warrant held by the Investor to purchase 2,920,306 shares of common stock of Acer Therapeutics Inc. The shares of common stock and warrants were offered and sold to the Investor in a registered direct offering without an underwriter or placement agent.

Registration Rights

Demand Registration Rights

OTA has the right to demand that we file a Form S-1 registration statement, as long as the anticipated aggregate offering price, net of underwriting discounts and commissions, would exceed $15.0 million. These registration rights are subject to specified conditions and limitations, including the right of the underwriters, if any, to limit the number of shares included in any such registration under specified circumstances. Upon such a request, we are required to effect the registration as soon as reasonably possible.

Registration on Form S-3

OTA is entitled, upon its written request, to have such shares registered by us on a Form S-3 registration statement at our expense, subject to other specified conditions and limitations.

Expenses of Registration

We will pay all expenses relating to any demand, piggyback or Form S-3 registration, other than underwriting discounts and commissions, subject to specified conditions and limitations.

Termination of Registration Rights

The registration rights granted under the investors' rights agreement terminated as to all the holders of our capital stock, other than OTA, on the two-year anniversary of our initial public offering. These registration rights will terminate as to OTA upon the earliest to occur of (i) written consent of OTA, (ii) such time that the OTA Warrant has been exercised in full and Rule 144 or another similar exemption under the Securities Act is available for the sale of all shares of our capital stock held by OTA without limitation during a three-month period without registration or (iii) six-months following the expiration of the OTA Warrant.

Registration Rights Agreement

On August 30, 2023, Zevra purchased certain indebtedness of Acer held by Nantahala Capital Management, LLC ("NCM"), certain of its affiliates and certain other parties (collectively with NCM, "Nantahala") pursuant to a Loan Purchase Agreement and a Note Purchase Agreement. In connection with entering into the Loan and Note Purchase Agreements, Zevra and Nantahala concurrently entered into a registration rights agreement (the "Registration Rights Agreement"), pursuant to which Zevra agreed to file a resale registration statement with respect to the resale of the Zevra common stock issuable under the Loan and Note Purchase Agreements and the Nantahala Note. On February 5, 2024, Zevra filed a registration statement on Form S-3 (File No. 333-276856) registering an aggregate of 2,269,721 shares of Zevra's common stock that were issued pursuant to the Loan and Note Purchase Agreements

Anti-Takeover Provisions

Section 203 of the Delaware General Corporation Law

We are subject to Section 203 of the Delaware General Corporation Law, which prohibits a Delaware corporation from engaging in any business combination with any interested stockholder for a period of three years after the date that such stockholder became an interested stockholder, with the following exceptions:

- before such date, the board of directors of the corporation approved either the business combination or the transaction that resulted in the stockholder becoming an interested stockholder;
- upon completion of the transaction that resulted in the stockholder becoming an interested stockholder, the interested stockholder owned at least 85% of the voting stock of the corporation outstanding at the time the transaction began, excluding for purposes of determining the voting stock outstanding, but not the outstanding voting stock owned by the interested stockholder, those shares owned (i) by persons who are directors and also officers and (ii) employee stock plans in which employee participants do not have the right to determine confidentially whether shares held subject to the plan will be tendered in a tender or exchange offer; or
- on or after such date, the business combination is approved by the board of directors and authorized at an annual or special meeting of the stockholders, and not by written consent, by the affirmative vote of at least 66 2/3% of the outstanding voting stock that is not owned by the interested stockholder.

In general, Section 203 defines a "business combination" to include the following:

- any merger or consolidation involving the corporation and the interested stockholder;
- any sale, transfer, pledge or other disposition of 10% or more of the assets of the corporation involving the interested stockholder;
- subject to certain exceptions, any transaction that results in the issuance or transfer by the corporation of any stock of the corporation to the interested stockholder;
- any transaction involving the corporation that has the effect of increasing the proportionate share of the stock or any class or series of the corporation beneficially owned by the interested stockholder; or
- the receipt by the interested stockholder of the benefit of any loans, advances, guarantees, pledges or other financial benefits by or through the corporation.

In general, Section 203 defines an "interested stockholder" as an entity or person who, together with the person's affiliates and associates, beneficially owns, or within three years prior to the time of determination of interested stockholder status did own, 15% or more of the outstanding voting stock of the corporation.

Certificate of Incorporation and Bylaws

Our amended and restated certificate of incorporation provides for our board of directors to be divided into three classes with staggered three-year terms. Only one class of directors will be elected at each annual meeting of our stockholders, with the other classes continuing for the remainder of their respective three-year terms. The affirmative vote of the majority (plurality, in the case of the election of directors) of shares present and entitled to vote generally on a subject matter shall be the act of the stockholders. Our amended and restated certificate of incorporation and our amended and restated bylaws also provide that directors may be removed by the stockholders only for cause upon the vote of 66 2/3% or more of our outstanding common stock. Furthermore, the authorized number of directors may be changed only by resolution of the board of directors, and vacancies and newly created directorships on the board of directors may, except as otherwise required by law or determined by the board of directors, only be filled by a majority vote of the directors then serving on the board of directors, even though less than a quorum.

Our amended and restated certificate of incorporation and amended and restated bylaws also provide that all stockholder actions must be effected at a duly called meeting of stockholders and eliminates the right of stockholders to act by written consent without a meeting. Our amended and restated bylaws also provide that only our chairman of the board, chief executive officer or the board of directors pursuant to a resolution adopted by a majority of the total number of authorized directors may call a special meeting of stockholders.

Our amended and restated bylaws also provide that stockholders seeking to present proposals before a meeting of stockholders or to nominate candidates for election as directors at a meeting of stockholders must provide timely advance notice in writing, and specify requirements as to the form and content of a stockholder's notice.

Our amended and restated certificate of incorporation and amended and restated bylaws provide that the stockholders cannot amend many of the provisions described above except by a vote of 66 2/3% or more of our outstanding common stock.

The combination of these provisions make it more difficult for our existing stockholders to replace our board of directors as well as for another party to obtain control of us by replacing our board of directors. Since our board of directors has the power to retain and discharge our officers, these provisions also make it more difficult for existing stockholders or another party to effect a change in management. In addition, the authorization of undesignated preferred stock makes it possible for our board of directors to issue preferred stock with voting or other rights or preferences that could impede the success of any attempt to change our control.

These provisions are intended to enhance the likelihood of continued stability in the composition of our board of directors and its policies and to discourage coercive takeover practices and inadequate takeover bids. These provisions are also designed to reduce our vulnerability to hostile takeovers and to discourage certain tactics that may be used in proxy fights. However, such provisions could have the effect of discouraging others from making tender offers for our shares and may have the effect of delaying changes in our control or management. As a consequence, these provisions may also inhibit fluctuations in the market price of our stock that could result from actual or rumored takeover attempts. We believe that the benefits of these provisions, including increased protection of our potential ability to negotiate with the proponent of an unfriendly or unsolicited proposal to acquire or restructure our company, outweigh the disadvantages of discouraging takeover proposals, because negotiation of takeover proposals could result in an improvement of their terms.

Choice of Forum

Our amended and restated bylaws provide that (1) unless we consent in writing to the selection of an alternative forum, the Court of Chancery of the State of Delaware (or, if and only if the Court of Chancery of the State of Delaware lacks subject matter jurisdiction, any state court located within the State of Delaware or, if and only if all such state courts lack subject matter jurisdiction, the federal district court for the District of Delaware) shall be the sole and exclusive forum for the following types of actions or proceedings under Delaware statutory or common law: (A) any derivative action or proceeding brought on behalf of us; (B) any action or proceeding asserting a claim of breach of a fiduciary duty owed by any current or former director, officer or other employee of us, to us or our stockholders; (C) any action or proceeding asserting a claim against us any current or former director, officer or other employee of us, arising out of or pursuant to any provision of the Delaware General Corporation Law, or the DGCL, our amended and restated certificate of incorporation or our amended and restated bylaws (as each may be amended from time to time); (D) any action or proceeding to interpret, apply, enforce or determine the validity of our amended and restated certificate of incorporation or our amended and restated bylaws (including any right, obligation, or remedy thereunder); (E) any action or proceeding as to which the DGCL confers jurisdiction to the Court of Chancery of the State of Delaware; and (F) any action or proceeding asserting a claim against us or any director, officer or other employee of us, governed by the internal affairs doctrine, in all cases to the fullest extent permitted by law and subject to the court's having personal jurisdiction over the indispensable parties named as defendants, provided that this provision shall not apply to suits brought to enforce a duty or liability created by the Securities Act or the Exchange Act or any other claim for which the federal courts have exclusive jurisdiction; (2) unless we consent in writing to the selection of an alternative forum, to the fullest extent permitted by law, the federal district courts of the United States of America shall be the exclusive forum for the resolution of any complaint asserting a cause of action arising under the Securities Act; and (3) any person or entity holding, owning or otherwise acquiring any interest in any of our securities shall be deemed to have notice of and consented to the provisions of our amended and restated bylaws.

Listing on the Nasdaq Stock Market

Our common stock is listed on the Nasdaq Global Select Market under the symbol "ZVRA."

ZEVRA THERAPEUTICS, INC.
2023 EMPLOYMENT INDUCEMENT AWARD PLAN
(AS AMENDED AND RESTATED FEBRUARY 22, 2024)

1. ***General.***

(a) Eligible Award Recipients. Eligible Individuals are eligible to receive Awards.

(b) Available Awards. The Plan provides for the grant of the following Awards: (i) Nonstatutory Stock Options, (ii) Stock Appreciation Rights (iii) Restricted Stock Awards, (iv) Restricted Stock Unit Awards, (v) Performance Stock Awards, (vi) Performance Cash Awards, and (vii) Other Stock Awards.

(c) Purpose. This Plan, through the granting of Awards, is intended to help the Company secure and retain the services of eligible award recipients, provide incentives for such persons to exert maximum efforts for the success of the Company and any Affiliate, and provide a means by which the eligible recipients may benefit from increases in value of the Common Stock. This Plan constitutes an amendment and restatement of the Company's 2023 Employment Inducement Award Plan.

2. ***Administration.***

(a) Administration by Board**.** The Board will administer the Plan. The Board may delegate administration of the Plan to a Committee or Committees, as provided in Section 2(c).

(b) Powers of Board. The Board will have the power, subject to, and within the limitations of, the express provisions of the Plan:

(i) To determine: (A) which Eligible Individuals will be granted Awards; (B) when and how each Award will be granted; (C) what type of Award will be granted; (D) the provisions of each Award (which need not be identical), including when a person will be permitted to exercise or otherwise receive cash or Common Stock under the Award; (E) the number of shares of Common Stock subject to, or the cash value of, an Award; and (F) the Fair Market Value applicable to a Stock Award.

(ii) To construe and interpret the Plan and Awards granted under it, and to establish, amend and revoke rules and regulations for administration of the Plan and Awards. The Board, in the exercise of these powers, may correct any defect, omission or inconsistency in the Plan or in any Award Agreement or in the written terms of a Performance Cash Award, in a manner and to the extent it will deem necessary or expedient to make the Plan or Award fully effective.

(iii) To settle all controversies regarding the Plan and Awards granted under it.

(iv) To accelerate, in whole or in part, the time at which an Award may be exercised or vest (or at which cash or shares of Common Stock may be issued).

(v) To suspend or terminate the Plan at any time. Except as otherwise provided in the Plan or an Award Agreement, suspension or termination of the Plan will not materially impair a Participant's rights under his or her then-outstanding Award without his or her written consent except as provided in subsection (viii) below.

(vi) To amend the Plan in any respect the Board deems necessary or advisable, including, without limitation, by adopting amendments relating to certain nonqualified deferred compensation under Section 409A of the Code and/or to bring the Plan or Awards granted under the Plan compliant with or exempt from the requirements for nonqualified deferred compensation under Section 409A of the Code, subject to the limitations, if any, of applicable law.

(vii) To adopt procedures from time to time that are intended to ensure that an individual is an Eligible Individual prior to the granting of any Awards to such individual (including without limitation a requirement that each such individual certify to the Company prior to the receipt of an Award that he or she is not currently employed by the Company or a Subsidiary and, if previously so employed, has had a bona fide period of interruption of employment, and that the grant of Awards is an inducement material to the Eligible Individual's agreement to enter into employment with the Company or a Subsidiary).

(viii) To approve forms of Award Agreements for use under the Plan and to amend the terms of any one or more Awards, including, but not limited to, amendments to provide terms more favorable to the Participant than previously provided in the Award Agreement, subject to any specified limits in the Plan that are not subject to Board discretion; *provided however,* that a Participant's rights under any Award will not be impaired by any such amendment unless (A) the Company requests the consent of the affected Participant, and (B) such Participant consents in writing. Notwithstanding the foregoing, (1) a Participant's rights will not be deemed to have been impaired by any such amendment if the Board, in its sole discretion, determines that the amendment, taken as a whole, does not materially impair the Participant's rights, and (2) subject to the limitations of applicable law, if any, the Board may amend the terms of any one or more Awards without the affected Participant's consent (A) to clarify the manner of exemption from, or to bring the Award into compliance with, Section 409A of the Code; or (B) to comply with other applicable laws or listing requirements.

(ix) Generally, to exercise such powers and to perform such acts as the Board deems necessary or expedient to promote the best interests of the Company and that are not in conflict with the provisions of the Plan or Awards.

(x) To adopt such procedures and sub-plans as are necessary or appropriate to permit participation in the Plan by Eligible Individuals who are foreign nationals or employed outside the United States (provided that Board approval will not be necessary for immaterial modifications to the Plan or any Award Agreement that are required for compliance with the laws of the relevant foreign jurisdiction).

(xi) To effect, with the consent of any adversely affected Participant, (A) the reduction of the exercise, purchase or strike price of any outstanding Stock Award; (B) the cancellation of any outstanding Stock Award and the grant in substitution therefor of a new (1) Option or SAR, (2) Restricted Stock Award, (3) Restricted Stock Unit Award, (4) Other Stock Award, (5) cash award and/or (6) award of other valuable consideration determined by the Board, in its sole discretion, with any such substituted award (x) covering the same or a different number of shares of Common Stock as the cancelled Stock Award and (y) granted under the Plan or another equity or compensatory plan of the Company; or (C) any other action that is treated as a repricing under generally accepted accounting principles.

(c) Delegation to Committee.

(i) *General***.** The Board may delegate some or all of the administration of the Plan to a Committee or Committees. If administration of the Plan is delegated to a Committee, the Committee will have, in connection with the administration of the Plan, the powers theretofore possessed by the Board that have been delegated to the Committee, including the power to delegate to a subcommittee of the Committee any of the administrative powers the Committee is authorized to exercise (and references in this Plan to the Board will thereafter be to the Committee or subcommittee, as applicable). Any delegation of administrative powers will be reflected in resolutions, not inconsistent with the provisions of the Plan, adopted from time to time by the Board or Committee (as applicable). The Board may retain the authority to concurrently administer the Plan with the Committee and may, at any time, revest in the Board some or all of the powers previously delegated.

(ii) *Rule 16b-3 Compliance*. To the extent an Award is intended to qualify for the exemption from Section 16(b) of the Exchange Act that is available under Rule 16b-3 of the Exchange Act, the Award will be granted by the Board or a Committee that consists solely of two or more Non-Employee Directors, as determined under Rule 16b-3(b)(3) of the Exchange Act and thereafter any action establishing or modifying the terms of the Award will be approved by the Board or a Committee, meeting such requirements to the extent necessary for such exemption to remain available.

(d) Effect of Board's Decision. All determinations, interpretations and constructions made by the Board or the Committee in good faith will not be subject to review by any person and will be final, binding and conclusive on all persons.

3. ***Shares Subject to the Plan.***

(a) Share Reserve. Subject to Section 9(a) relating to Capitalization Adjustments, the aggregate number of shares of Common Stock that may be issued pursuant to Stock Awards will not exceed 4,500,000 (the "***Share Reserve***"). For clarity, the Share Reserve is a limitation on the number of shares of Common Stock that may be issued under the Plan. Accordingly, this Section 3(a) does not limit the granting of Stock Awards except as provided in Section 7(a).

(b) Reversion of Shares to the Share Reserve. If a Stock Award or any portion thereof (i) expires or otherwise terminates without all of the shares covered by such Stock Award having been issued or (ii) is settled in cash (*i.e.*, the Participant receives cash rather than stock), such expiration, termination or settlement will not reduce (or otherwise offset) the number of shares of Common Stock that may be available for issuance under the Plan. If any shares of Common Stock issued pursuant to a Stock Award are forfeited back to or repurchased by the Company because of the failure to meet a contingency or condition required to vest such shares in the Participant, then the shares that are forfeited or repurchased will revert to and again become available for issuance under the Plan. Any shares reacquired by the Company in satisfaction of tax withholding obligations on a Stock Award or as consideration for the exercise or purchase price of a Stock Award will again become available for issuance under the Plan.

(c) Source of Shares. The stock issuable under the Plan will be shares of authorized but unissued or reacquired Common Stock, including shares repurchased by the Company on the open market or otherwise.

4. ***Eligibility for Specific Stock Awards.***

Stock Awards may be granted to Eligible Individuals.

5. ***Provisions Relating to Options and Stock Appreciation Rights.***

Each Option or SAR will be in such form and will contain such terms and conditions as the Board deems appropriate. All Options will be Nonstatutory Stock Options, and, if certificates are issued, a separate certificate or certificates will be issued for shares of Common Stock purchased on exercise of each Option. The provisions of separate Options or SARs need not be identical; *provided, however*, that each Award Agreement will conform to (through incorporation of provisions hereof by reference in the applicable Award Agreement or otherwise) the substance of each of the following provisions:

(a) Term. No Option or SAR will be exercisable after the expiration of ten years from the date of its grant or such shorter period specified in the Award Agreement.

(b) Exercise Price. The exercise or strike price of each Option or SAR will be not less than 100% of the Fair Market Value of the Common Stock subject to the Option or SAR on the date the Award is granted. Each SAR will be denominated in shares of Common Stock equivalents.

(c) Purchase Price for Options. The purchase price of Common Stock acquired pursuant to the exercise of an Option may be paid, to the extent permitted by applicable law and as determined by the Board in its sole discretion, by any combination of the methods of payment set forth below. The Board will have the authority to grant Options that do not permit all of the following methods of payment (or otherwise restrict the ability to use certain methods) and to grant Options that require the consent of the Company to use a particular method of payment. The permitted methods of payment are as follows:

(i) by cash, check, bank draft or money order payable to the Company;

(ii) pursuant to a program developed under Regulation T as promulgated by the Federal Reserve Board that, prior to the issuance of the stock subject to the Option, results in either the receipt of cash (or check) by the Company or the receipt of irrevocable instructions to pay the aggregate exercise price to the Company from the sales proceeds;

(iii) by delivery to the Company (either by actual delivery or attestation) of shares of Common Stock;

(iv) by a "net exercise" arrangement pursuant to which the Company will reduce the number of shares of Common Stock issuable upon exercise of an Option by the largest whole number of shares with a Fair Market Value that does not exceed the aggregate exercise price; *provided, however*, that the Company will accept a cash or other payment from the Participant to the extent of any remaining balance of the aggregate exercise price not satisfied by such reduction in the number of whole shares to be issued. Shares of Common Stock will no longer be subject to an Option and will not be exercisable thereafter to the extent that (A) shares issuable upon exercise are reduced to pay the exercise price pursuant to the "net exercise," (B) shares are delivered to the Participant as a result of such exercise, and (C) shares are withheld to satisfy tax withholding obligations; or

(v) in any other form of legal consideration that may be acceptable to the Board and specified in the applicable Award Agreement.

(d) Exercise and Payment of a SAR. To exercise any outstanding SAR, the Participant must provide written notice of exercise to the Company in compliance with the provisions of the Stock Appreciation Right Agreement evidencing such SAR. The appreciation distribution payable on the exercise of a SAR will be not greater than an amount equal to the excess of (A) the aggregate Fair Market Value (on the date of the exercise of the SAR) of a number of shares of Common Stock equal to the number of Common Stock equivalents in which the Participant is vested under such SAR, and with respect to which the Participant is exercising the SAR on such date, over (B) the aggregate strike price of the number of Common Stock equivalents with respect to which the Participant is exercising the SAR on such date. The appreciation distribution may be paid in Common Stock, in cash, in any combination of the two or in any other form of consideration, as determined by the Board and contained in the Award Agreement evidencing such SAR.

(e) Transferability of Options and SARs. The Board may, in its sole discretion, impose such limitations on the transferability of Options and SARs as the Board will determine. In the absence of such a determination by the Board to the contrary, the following restrictions on the transferability of Options and SARs will apply:

(i) *Restrictions on Transfer*. An Option or SAR will not be transferable except by will or by the laws of descent and distribution (or pursuant to subsections (ii) and (iii) below), and will be exercisable during the lifetime of the Participant only by the Participant. The Board may permit transfer of the Option or SAR in a manner that is not prohibited by applicable tax and securities laws. Except as explicitly provided herein, neither an Option nor a SAR may be transferred for consideration.

(ii) *Domestic Relations Orders*. Subject to the approval of the Board or a duly authorized Officer, an Option or SAR may be transferred pursuant to the terms of a domestic relations order, official marital settlement agreement.

(iii) *Beneficiary Designation*. Subject to the approval of the Board or a duly authorized Officer, a Participant may, by delivering written notice to the Company, in a form approved by the Company (or the designated broker), designate a third party who, on the death of the Participant, will thereafter be entitled to exercise the Option or SAR and receive the Common Stock or other consideration resulting from such exercise. In the absence of such a designation, the executor or administrator of the Participant's estate will be entitled to exercise the Option or SAR and receive the Common Stock or other consideration resulting from such exercise. However, the Company may prohibit designation of a beneficiary at any time, including due to any conclusion by the Company that such designation would be inconsistent with the provisions of applicable laws.

(f) Vesting Generally. The total number of shares of Common Stock subject to an Option or SAR may vest and become exercisable in periodic installments that may or may not be equal. The Option or SAR may be subject to such other terms and conditions on the time or times when it may or may not be exercised (which may be based on the satisfaction of Performance Goals or other criteria) as the Board may deem appropriate. The vesting provisions of individual Options or SARs may vary. The provisions of this Section 5(f) are subject to any Option or SAR provisions governing the minimum number of shares of Common Stock as to which an Option or SAR may be exercised.

(g) Termination of Continuous Service. Except as otherwise provided in the applicable Award Agreement or other agreement between the Participant and the Company, if a Participant's Continuous Service terminates (other than for Cause and other than upon the Participant's death or Disability), the Participant may exercise his or her Option or SAR (to the extent that the Participant was entitled to exercise such Award as of the date of termination of Continuous Service) within the period of time ending on the earlier of (i) the date three months following the termination of the Participant's Continuous Service (or such longer or shorter period specified in the applicable Award Agreement), and (ii) the expiration of the term of the Option or SAR as set forth in the Award Agreement. If, after termination of Continuous Service, the Participant does not exercise his or her Option or SAR (as applicable) within the applicable time frame, the Option or SAR will terminate.

(h) Extension of Termination Date. If the exercise of an Option or SAR following the termination of the Participant's Continuous Service (other than for Cause and other than upon the Participant's death or Disability) would be prohibited at any time solely because the issuance of shares of Common Stock would violate the registration requirements under the Securities Act, then the Option or SAR will terminate on the earlier of (i) the expiration of a total period of time (that need not be consecutive) equal to the applicable post termination exercise period after the termination of the Participant's Continuous Service during which the exercise of the Option or SAR would not be in violation of such registration requirements, and (ii) the expiration of the term of the Option or SAR as set forth in the applicable Award Agreement. In addition, unless otherwise provided in a Participant's Award Agreement, if the sale of any Common Stock received on exercise of an Option or SAR following the termination of the Participant's Continuous Service (other than for Cause) would violate the Company's insider trading policy, then the Option or SAR will terminate on the earlier of (i) the expiration of a period of months (that need not be consecutive) equal to the applicable post-termination exercise period after the termination of the Participant's Continuous Service during which the sale of the Common Stock received upon exercise of the Option or SAR would not be in violation of the Company's insider trading policy, or (ii) the expiration of the term of the Option or SAR as set forth in the applicable Award Agreement.

(i) Disability of Participant. Except as otherwise provided in the applicable Award Agreement or other agreement between the Participant and the Company, if a Participant's Continuous Service terminates as a result of the Participant's Disability, the Participant may exercise his or her Option or SAR (to the extent that the Participant was entitled to exercise such Option or SAR as of the date of termination of Continuous Service), but only within such period of time ending on the earlier of (i) the date 12 months following such termination of Continuous Service (or such longer or shorter period specified in the Award Agreement), and (ii) the expiration of the term of the Option or SAR as set forth in the Award Agreement. If, after termination of Continuous Service, the Participant does not exercise his or her Option or SAR within the applicable time frame, the Option or SAR (as applicable) will terminate.

(j) Death of Participant. Except as otherwise provided in the applicable Award Agreement or other agreement between the Participant and the Company, if (i) a Participant's Continuous Service terminates as a result of the Participant's death, or (ii) the Participant dies within the period (if any) specified in the Award Agreement for exercisability after the termination of the Participant's Continuous Service for a reason other than death, then the Option or SAR may be exercised (to the extent the Participant was entitled to exercise such Option or SAR as of the date of death) by the Participant's estate, by a person who acquired the right to exercise the Option or SAR by bequest or inheritance or by a person designated to exercise the Option or SAR upon the Participant's death, but only within the period ending on the earlier of (i) the date 18 months following the date of death (or such longer or shorter period specified in the Award Agreement), and (ii) the expiration of the term of such Option or SAR as set forth in the Award Agreement. If, after the Participant's death, the Option or SAR is not exercised within the applicable time frame, the Option or SAR (as applicable) will terminate.

(k) Termination for Cause. Except as explicitly provided otherwise in a Participant's Award Agreement or other individual written agreement between the Company or any Affiliate and the Participant, if a Participant's Continuous Service is terminated for Cause, the Option or SAR will terminate immediately upon such Participant's termination of Continuous Service, and the Participant will be prohibited from exercising his or her Option or SAR from and after the date of such termination of Continuous Service.

(l) Non-Exempt Employees. If an Option or SAR is granted to an Employee who is a non-exempt employee for purposes of the Fair Labor Standards Act of 1938, as amended, the Option or SAR will not be first exercisable for any shares of Common Stock until at least six months following the date of grant of the Option or SAR (although the Award may vest prior to such date). Consistent with the provisions of the Worker Economic Opportunity Act, (i) if such non-exempt Employee dies or suffers a Disability, (ii) upon a Corporate Transaction in which such Option or SAR is not assumed, continued, or substituted, (iii) upon a Change in Control, or (iv) upon the Participant's retirement (as such term may be defined in the Participant's Award Agreement in another agreement between the Participant and the Company, or, if no such definition, in accordance with the Company's then current employment policies and guidelines), the vested portion of any Options and SARs may be exercised earlier than six months following the date of grant. The foregoing provision is intended to operate so that any income derived by a non-exempt employee in connection with the exercise or vesting of an Option or SAR will be exempt from his or her regular rate of pay. To the extent permitted and/or required for compliance with the Worker Economic Opportunity Act to ensure that any income derived by a non-exempt employee in connection with the exercise, vesting or issuance of any shares under any other Stock Award will be exempt from the employee's regular rate of pay, the provisions of this Section 5(l) will apply to all Stock Awards and are hereby incorporated by reference into such Stock Award Agreements.

6. ***Provisions of Stock Awards other than Options and SARs.***

(a) Restricted Stock Awards. Each Restricted Stock Award Agreement will be in such form and will contain such terms and conditions as the Board will deem appropriate. To the extent consistent with the Company's bylaws, at the Board's election, shares of Common Stock may be (x) held in book entry form subject to the Company's instructions until any restrictions relating to the Restricted Stock Award lapse; or (y) evidenced by a certificate, which certificate will be held in such form and manner as determined by the Board. The terms and conditions of Restricted Stock Award Agreements may change from time to time, and the terms and conditions of separate Restricted Stock Award Agreements need not be identical. Each Restricted Stock Award Agreement will conform to (through incorporation of the provisions hereof by reference in the agreement or otherwise) the substance of each of the following provisions:

(i) *Consideration*. A Restricted Stock Award may be awarded in consideration for (A) cash, check, bank draft or money order payable to the Company, (B) past services to the Company or an Affiliate, or (C) any other form of legal consideration (including future services) that may be acceptable to the Board, in its sole discretion, and permissible under applicable law.

(ii) *Vesting*. Shares of Common Stock awarded under the Restricted Stock Award Agreement may be subject to forfeiture to the Company in accordance with a vesting schedule to be determined by the Board.

(iii) *Termination of Participant's Continuous Service*. If a Participant's Continuous Service terminates, the Company may receive through a forfeiture condition or a repurchase right any or all of the shares of Common Stock held by the Participant as of the date of termination of Continuous Service under the terms of the Restricted Stock Award Agreement.

(iv) *Transferability*. Rights to acquire shares of Common Stock under the Restricted Stock Award Agreement will be transferable by the Participant only upon such terms and conditions as are set forth in the Restricted Stock Award Agreement, as the Board will determine in its sole discretion, so long as Common Stock awarded under the Restricted Stock Award Agreement remains subject to the terms of the Restricted Stock Award Agreement.

(v) *Dividends*. A Restricted Stock Award Agreement may provide that any dividends paid on Restricted Stock will be subject to the same vesting and forfeiture restrictions as apply to the shares subject to the Restricted Stock Award to which they relate.

(b) Restricted Stock Unit Awards. Each Restricted Stock Unit Award Agreement will be in such form and will contain such terms and conditions as the Board will deem appropriate. The terms and conditions of Restricted Stock Unit Award Agreements may change from time to time, and the terms and conditions of separate Restricted Stock Unit Award Agreements need not be identical. Each Restricted Stock Unit Award Agreement will conform to (through incorporation of the provisions hereof by reference in the Agreement or otherwise) the substance of each of the following provisions:

(i) *Consideration*. At the time of grant of a Restricted Stock Unit Award, the Board will determine the consideration, if any, to be paid by the Participant upon delivery of each share of Common Stock subject to the Restricted Stock Unit Award. The consideration to be paid (if any) by the Participant for each share of Common Stock subject to a Restricted Stock Unit Award may be paid in any form of legal consideration that may be acceptable to the Board, in its sole discretion, and permissible under applicable law.

(ii) *Vesting*. At the time of the grant of a Restricted Stock Unit Award, the Board may impose such restrictions on or conditions to the vesting of the Restricted Stock Unit Award as it, in its sole discretion, deems appropriate.

(iii) *Payment*. A Restricted Stock Unit Award may be settled by the delivery of shares of Common Stock, their cash equivalent, any combination thereof or in any other form of consideration, as determined by the Board and contained in the Restricted Stock Unit Award Agreement.

(iv) *Additional Restrictions*. At the time of the grant of a Restricted Stock Unit Award, the Board, as it deems appropriate, may impose such restrictions or conditions that delay the delivery of the shares of Common Stock (or their cash equivalent) subject to a Restricted Stock Unit Award to a time after the vesting of such Restricted Stock Unit Award.

(v) *Dividend Equivalents*. Dividend equivalents may be credited in respect of shares of Common Stock covered by a Restricted Stock Unit Award, as determined by the Board and contained in the Restricted Stock Unit Award Agreement. At the sole discretion of the Board, such dividend equivalents may be converted into additional shares of Common Stock covered by the Restricted Stock Unit Award in such manner as determined by the Board. Any additional shares covered by the Restricted Stock Unit Award credited by reason of such dividend equivalents will be subject to all of the same terms and conditions of the underlying Restricted Stock Unit Award Agreement to which they relate.

(vi) *Termination of Participant's Continuous Service*. Except as otherwise provided in the applicable Restricted Stock Unit Award Agreement, such portion of the Restricted Stock Unit Award that has not vested will be forfeited upon the Participant's termination of Continuous Service.

(c) Performance Awards.

(i) *Performance Stock Awards*. A Performance Stock Award is a Stock Award that is payable (including that may be granted, may vest or may be exercised) contingent upon the attainment during a Performance Period of certain Performance Goals. A Performance Stock Award may, but need not, require the Participant's completion of a specified period of Continuous Service. The length of any Performance Period, the Performance Goals to be achieved during the Performance Period, and the measure of whether and to what degree such Performance Goals have been attained will be conclusively determined by the Committee or the Board, in its sole discretion. In addition, to the extent permitted by applicable law and the applicable Award Agreement, the Board may determine that cash may be used in payment of Performance Stock Awards.

(ii) *Performance Cash Award*s. A Performance Cash Award is a cash award that is payable contingent upon the attainment during a Performance Period of certain Performance Goals. A Performance Cash Award may also require the completion of a specified period of Continuous Service. At the time of grant of a Performance Cash Award, the length of any Performance Period, the Performance Goals to be achieved during the Performance Period, and the measure of whether and to what degree such Performance Goals have been attained will be conclusively determined by the Committee or the Board, in its sole discretion. The Board may specify the form of payment of Performance Cash Awards, which may be cash or other property, or may provide for a Participant to have the option for his or her Performance Cash Award, or such portion thereof as the Board may specify, to be paid in whole or in part in cash or other property.

(iii) *Board Discretion*. The Board retains the discretion to reduce or eliminate the compensation or economic benefit due upon attainment of Performance Goals and to define the manner of calculating the Performance Criteria it selects to use for a Performance Period.

(d) Other Stock Awards. Other forms of Stock Awards valued in whole or in part by reference to, or otherwise based on, Common Stock, including the appreciation in value thereof (e.g., options or stock rights with an exercise price or strike price less than 100% of the Fair Market Value of the Common Stock at the time of grant) may be granted either alone or in addition to Stock Awards provided for under Section 5 and the preceding provisions of this Section 6. Subject to the provisions of the Plan, the Board will have sole and complete authority to determine the persons to whom and the time or times at which such Other Stock Awards will be granted, the number of shares of Common Stock (or the cash equivalent thereof) to be granted pursuant to such Other Stock Awards and all other terms and conditions of such Other Stock Awards.

7. ***Covenants of the Company.***

(a) Availability of Shares. The Company will keep available at all times the number of shares of Common Stock reasonably required to satisfy then-outstanding Awards.

(b) Securities Law Compliance. The Company will seek to obtain from each regulatory commission or agency having jurisdiction over the Plan such authority as may be required to grant Stock Awards and to issue and sell shares of Common Stock upon exercise of the Stock Awards; *provided, however*, that this undertaking will not require the Company to register under the Securities Act the Plan, any Stock Award or any Common Stock issued or issuable pursuant to any such Stock Award. If, after reasonable efforts and at a reasonable cost, the Company is unable to obtain from any such regulatory commission or agency the authority that counsel for the Company deems necessary for the lawful issuance and sale of Common Stock under the Plan, the Company will be relieved from any liability for failure to issue and sell Common Stock upon exercise of such Stock Awards unless and until such authority is obtained. A Participant will not be eligible for the grant of an Award or the subsequent issuance of cash or Common Stock pursuant to the Award if such grant or issuance would be in violation of any applicable securities law.

(c) No Obligation to Notify or Minimize Taxes. The Company will have no duty or obligation to any Participant to advise such holder as to the time or manner of exercising such Stock Award. Furthermore, the Company will have no duty or obligation to warn or otherwise advise such holder of a pending termination or expiration of an Award or a possible period in which the Award may not be exercised. The Company has no duty or obligation to minimize the tax consequences of an Award to the holder of such Award.

8. ***Miscellaneous.***

(a) Use of Proceeds from Sales of Common Stock. Proceeds from the sale of shares of Common Stock pursuant to Awards will constitute general funds of the Company.

(b) Corporate Action Constituting Grant of Awards. Corporate action constituting a grant by the Company of an Award to any Participant will be deemed completed as of the date of such corporate action, unless otherwise determined by the Board, regardless of when the instrument, certificate, or letter evidencing the Award is communicated to, or actually received or accepted by, the Participant. In the event that the corporate records (e.g., Board consents, resolutions or minutes) documenting the corporate action constituting the grant contain terms (e.g., exercise price, vesting schedule or number of shares) that are inconsistent with those in the Award Agreement or related grant documents as a result of a clerical error in the papering of the Award Agreement or related grant documents, the corporate records will control and the Participant will have no legally binding right to the incorrect term in the Award Agreement or related grant documents.

(c) Stockholder Rights. No Participant will be deemed to be the holder of, or to have any of the rights of a holder with respect to, any shares of Common Stock subject to an Award unless and until (i) such Participant has satisfied all requirements for exercise of, or the issuance of shares under, the Award pursuant to its terms, and (ii) the issuance of the Common Stock subject to such Award has been entered into the books and records of the Company.

(d) No Employment or Other Service Rights. Nothing in the Plan, any Award Agreement or any other instrument executed thereunder or in connection with any Award granted pursuant thereto will confer upon any Participant any right to continue to serve the Company or an Affiliate in the capacity in effect at the time the Award was granted or will affect the right of the Company or an Affiliate to terminate (i) the employment of an Employee with or without notice and with or without cause, (ii) the service of a Consultant pursuant to the terms of such Consultant's agreement with the Company or an Affiliate, or (iii) the service of a Director pursuant to the bylaws of the Company or an Affiliate, and any applicable provisions of the corporate law of the state in which the Company or the Affiliate is incorporated, as the case may be.

(e) Change in Time Commitment. In the event a Participant's regular level of time commitment in the performance of his or her services for the Company and any Affiliates is reduced (for example, and without limitation, if the Participant is an Employee of the Company and the Employee has a change in status from a full-time Employee to a part-time Employee or takes an extended leave of absence) after the date of grant of any Award to the Participant, the Board has the right in its sole discretion to (x) make a corresponding reduction in the number of shares or cash amount subject to any portion of such Award that is scheduled to vest or become payable after the date of such change in time commitment, and (y) in lieu of or in combination with such a reduction, extend the vesting or payment schedule applicable to such Award. In the event of any such reduction or extension, the Participant will have no right with respect to any portion of the Award that is so reduced or extended.

(f) Investment Assurances. The Company may require a Participant, as a condition of exercising or acquiring Common Stock under any Award, (i) to give written assurances satisfactory to the Company as to the Participant's knowledge and experience in financial and business matters and/or to employ a purchaser representative reasonably satisfactory to the Company who is knowledgeable and experienced in financial and business matters and that he or she is capable of evaluating, alone or together with the purchaser representative, the merits and risks of exercising the Award; and (ii) to give written assurances satisfactory to the Company stating that the Participant is acquiring Common Stock subject to the Award for the Participant's own account and not with any present intention of selling or otherwise distributing the Common Stock. The foregoing requirements, and any assurances given pursuant to such requirements, will be inoperative if (A) the issuance of the shares upon the exercise or acquisition of Common Stock under the Award has been registered under a then currently effective registration statement under the Securities Act, or (B) as to any particular requirement, a determination is made by counsel for the Company that such requirement need not be met in the circumstances under the then applicable securities laws. The Company may, upon advice of counsel to the Company, place legends on stock certificates issued under the Plan as such counsel deems necessary or appropriate in order to comply with applicable securities laws, including, but not limited to, legends restricting the transfer of the Common Stock.

(g) Withholding Obligations. Unless prohibited by the terms of an Award Agreement, the Company or any subsidiary may, in its sole discretion, satisfy any federal, state or local tax withholding obligation relating to an Award by any of the following means or by a combination of such means: (i) causing the Participant to tender a cash payment; (ii) withholding shares of Common Stock from the shares of Common Stock issued or otherwise issuable to the Participant in connection with the Award; *provided, however,* that no shares of Common Stock are withheld with a value exceeding the amount of tax required to be withheld by law based on the minimum statutory withholding rates (or such other rate, not to exceed the maximum statutory withholding rate, as may be determined by the Company after considering any accounting consequences or costs); (iii) withholding cash from an Award settled in cash; (iv) withholding payment from any amounts otherwise payable to the Participant; or (v) by such other method as may be set forth in the Award Agreement.

(h) Electronic Delivery. Any reference herein to a "written" agreement or document will include any agreement or document delivered electronically, filed publicly at www.sec.gov (or any successor website thereto) or posted on the Company's intranet (or other shared electronic medium controlled by the Company to which the Participant has access).

(i) Deferrals. To the extent permitted by applicable law, the Board, in its sole discretion, may determine that the delivery of Common Stock or the payment of cash, upon the exercise, vesting or settlement of all or a portion of any Award may be deferred and may establish programs and procedures for deferral elections to be made by Participants. Deferrals by Participants will be made in accordance with Section 409A of the Code. Consistent with Section 409A of the Code, the Board may provide for distributions while a Participant is still an employee or otherwise providing services to the Company. The Board is authorized to make deferrals of Awards and determine when, and in what annual percentages, Participants may receive payments, including lump sum payments, following the Participant's termination of Continuous Service, and implement such other terms and conditions consistent with the provisions of the Plan and in accordance with applicable law.

(j) Compliance with Section 409A. Unless otherwise expressly provided for in an Award Agreement, the Plan and Award Agreements will be interpreted to the greatest extent possible in a manner that makes the Plan and the Awards granted hereunder exempt from Section 409A of the Code, and, to the extent not so exempt, in compliance with Section 409A of the Code. If the Board determines that any Award granted hereunder is not exempt from and is therefore subject to Section 409A of the Code, the Award Agreement evidencing such Award will incorporate the terms and conditions necessary to avoid the consequences specified in Section 409A(a)(1) of the Code, and to the extent an Award Agreement is silent on terms necessary for compliance, such terms are hereby incorporated by reference into the Award Agreement. Notwithstanding anything to the contrary in this Plan (and unless the Award Agreement specifically provides otherwise), if the shares of Common Stock are publicly traded, and if a Participant holding an Award that constitutes "deferred compensation" under Section 409A of the Code is a "specified employee" for purposes of Section 409A of the Code, no distribution or payment of any amount that is due because of a "separation from service" (as defined in Section 409A of the Code without regard to alternative definitions thereunder) will be issued or paid before the date that is six months following the date of such Participant's "separation from service" or, if earlier, the date of the Participant's death, unless such distribution or payment can be made in a manner that complies with Section 409A of the Code, and any amounts so deferred will be paid in a lump sum on the day after such six month period elapses, with the balance paid thereafter on the original schedule.

(k) Clawback/Recovery. All Awards (including, without limitation, any proceeds, gains or other economic benefit actually or constructively received by a Participant upon any receipt or exercise of the Award or upon the receipt or sale of any shares of Common Stock underlying the Award) granted under the Plan will be subject to recoupment in accordance with any clawback policy that the Company is required to adopt pursuant to the listing standards of any national securities exchange or association on which the Company's securities are listed or as is otherwise required by the Dodd-Frank Wall Street Reform and Consumer Protection Act or other applicable law (including, without limitation, the Company's Policy for Recovery of Erroneously Awarded Compensation). In addition, the Board may impose such other clawback, recovery or recoupment provisions, including in an Award Agreement, as the Board determines necessary or appropriate, including but not limited to a reacquisition right in respect of previously acquired shares of Common Stock or other cash or property upon the occurrence of an event constituting Cause. No recovery of compensation under such a clawback policy will be an event giving rise to a right to resign for "good reason" or "constructive termination" (or similar term) under any agreement with the Company.

(l) Action Required Upon Grant of Award. Promptly following the grant of an Award, the Company shall, in accordance with NASDAQ Rule 5635(c), (i) issue a press release disclosing the material terms of the Award, including the recipient(s) of the Award and the number of Shares involved and (ii) provide written notice to NASDAQ of the grant.

(m) Stockholder Approval Not Required. It is expressly intended that approval of the Company's stockholders not be required as a condition of the effectiveness of the Plan, and the Plan's provisions shall be interpreted in a manner consistent with such intent for all purposes. Specifically, NASDAQ Rule 5635(c) generally requires stockholder approval for equity compensation plans adopted by companies whose securities are listed on the NASDAQ Stock Market that provide for the delivery of equity securities to any employees, directors or other service providers of such companies as compensation for services. NASDAQ Rule 5635(c)(4) provides an exemption in certain circumstances for employment inducement awards. Notwithstanding anything to the contrary herein, in accordance with NASDAQ Rule 5635(c)(4), Awards may only be granted as material inducements to Eligible Individuals being hired or rehired as Employees, as applicable, and must be approved by (i) the Board, acting through a majority of the Company's Independent Directors or (ii) the independent Compensation Committee of the Board. Accordingly, pursuant to NASDAQ Rule 5635(c)(4), the issuance of Awards and the Shares issuable upon exercise or vesting of such Awards pursuant to the Plan is not subject to the approval of the Company's stockholders.

9. ***Adjustments upon Changes in Common Stock; Other Corporate Events.***

(a) Capitalization Adjustments. In the event of a Capitalization Adjustment, the Board will appropriately and proportionately adjust: (i) the class(es) and maximum number of securities subject to the Plan pursuant to Section 3(a) and (ii) the class(es) and number of securities and price per share of stock subject to outstanding Stock Awards. The Board will make such adjustments, and its determination will be final, binding and conclusive.

(b) Dissolution or Liquidation. Except as otherwise provided in the Stock Award Agreement, in the event of a dissolution or liquidation of the Company, all outstanding Stock Awards (other than Stock Awards consisting of vested and outstanding shares of Common Stock not subject to a forfeiture condition or the Company's right of repurchase) will terminate immediately prior to the completion of such dissolution or liquidation, and the shares of Common Stock subject to the Company's repurchase rights or subject to a forfeiture condition may be repurchased or reacquired by the Company notwithstanding the fact that the holder of such Stock Award is providing Continuous Service; *provided, however*, that the Board may, in its sole discretion, cause some or all Stock Awards to become fully vested, exercisable and/or no longer subject to repurchase or forfeiture (to the extent such Stock Awards have not previously expired or terminated) before the dissolution or liquidation is completed but contingent on its completion.

(c) Corporate Transaction. The following provisions will apply to Stock Awards in the event of a Corporate Transaction unless otherwise provided in the instrument evidencing the Stock Award or any other written agreement between the Company or any Affiliate and the Participant or unless otherwise expressly provided by the Board at the time of grant of a Stock Award. In the event of a Corporate Transaction, then, notwithstanding any other provision of the Plan, the Board will take one or more of the following actions with respect to Stock Awards, contingent upon the closing or completion of the Corporate Transaction:

(i) arrange for the surviving corporation or acquiring corporation (or the surviving or acquiring corporation's parent company) to assume or continue the Stock Award or to substitute a similar stock award for the Stock Award (including, but not limited to, an award to acquire the same consideration paid to the stockholders of the Company pursuant to the Corporate Transaction);

(ii) arrange for the assignment of any reacquisition or repurchase rights held by the Company in respect of Common Stock issued pursuant to the Stock Award to the surviving corporation or acquiring corporation (or the surviving or acquiring corporation's parent company);

(iii) accelerate the vesting, in whole or in part, of the Stock Award (and, if applicable, the time at which the Stock Award may be exercised) to a date prior to the effective time of such Corporate Transaction as the Board will determine (or, if the Board will not determine such a date, to the date that is five days prior to the effective date of the Corporate Transaction), with such Stock Award terminating if not exercised (if applicable) at or prior to the effective time of the Corporate Transaction;

(iv) arrange for the lapse, in whole or in part, of any reacquisition or repurchase rights held by the Company with respect to the Stock Award;

(v) cancel or arrange for the cancellation of the Stock Award, to the extent not vested or not exercised prior to the effective time of the Corporate Transaction, in exchange for such cash consideration, if any, as the Board, in its sole discretion, may consider appropriate; and

(vi) make a payment, in such form as may be determined by the Board equal to the excess, if any, of (A) the value of the property the Participant would have received upon the exercise of the Stock Award immediately prior to the effective time of the Corporate Transaction, over (B) any exercise price payable by such holder in connection with such exercise.

The Board need not take the same action or actions with respect to all Stock Awards or portions thereof or with respect to all Participants. The Board may take different actions with respect to the vested and unvested portions of a Stock Award.

(d) Change in Control. A Stock Award may be subject to additional acceleration of vesting and exercisability upon or after a Change in Control as may be provided in the Stock Award Agreement for such Stock Award or as may be provided in any other written agreement between the Company or any Affiliate and the Participant, but in the absence of such provision, no such acceleration will occur.

10. ***Plan Term; Earlier Termination or Suspension of the Plan.***

The Board may suspend or terminate the Plan at any time. No Awards may be granted under the Plan while the Plan is suspended or after it is terminated.

11. ***Effective Date of the Plan.***

The Plan, as amended and restated, will become effective on the Amendment Date. For the avoidance of doubt, any Awards granted prior to the Amendment Date shall remain subject to the terms of the Award Agreement governing such Award, and the effectiveness of this Plan shall not constitute an amendment of such Award Agreement.

12. ***Choice of Law.***

The law of the State of Delaware will govern all questions concerning the construction, validity and interpretation of the Plan, without regard to that state's conflict of laws rules.

13. ***Definitions.***

As used in the Plan, the following definitions will apply to the capitalized terms indicated below:

(a) "***Affiliate***" means, at the time of determination, any "parent" or "subsidiary" of the Company as such terms are defined in Rule 405 of the Securities Act. The Board will have the authority to determine the time or times at which "parent" or "subsidiary" status is determined within the foregoing definition.

(b) "***Amendment Date***" means February 22, 2024.

(c) "***Award***" means a Stock Award or a Performance Cash Award.

(d) "***Award Agreement***" means a written or electronic agreement between the Company and a Participant evidencing the terms and conditions of an Award. Each Award Agreement will be subject to the terms and conditions of the Plan.

(e) "***Board***" means the Board of Directors of the Company.

(f) "***Capital Stock***" means each and every class of common stock of the Company, regardless of the number of votes per share.

(g) "***Capitalization Adjustment***" means any change that is made in, or other events that occur with respect to, the Common Stock subject to the Plan or subject to any Stock Award after the Amendment Date without the receipt of consideration by the Company through merger, consolidation, reorganization, recapitalization, reincorporation, stock dividend, dividend in property other than cash, large nonrecurring cash dividend, stock split, reverse stock split, liquidating dividend, combination of shares, exchange of shares, change in corporate structure or any similar equity restructuring transaction, as that term is used in Statement of Financial Accounting Standards Board Accounting Standards Codification Topic 718 (or any successor thereto). Notwithstanding the foregoing, the conversion of any convertible securities of the Company will not be treated as a Capitalization Adjustment.

(h) "***Cause***" will have the meaning ascribed to such term in any written agreement between the Participant and the Company defining such term and, in the absence of such agreement, such term shall mean, with respect to a Participant, the occurrence of any of the following events that has a material negative impact on the business or reputation of the Company: (i) such Participant's attempted commission of, or participation in, a fraud or act of dishonesty against the Company; (ii) such Participant's intentional, material violation of any contract or agreement between the Participant and the Company or of any statutory duty owed to the Company; (iii) such Participant's unauthorized use or disclosure of the Company's confidential information or trade secrets; or (iv) such Participant's gross misconduct. The determination that a termination of the Participant's Continuous Service is either for Cause or without Cause shall be made by the Company, in its sole discretion. Any determination by the Company that the Continuous Service of a Participant was terminated with or without Cause for the purposes of outstanding Awards held by such Participant shall have no effect upon any determination of the rights or obligations of the Company or such Participant for any other purpose.

(i) "***Change in Control***" means the occurrence, in a single transaction or in a series of related transactions, of any one or more of the following events:

(i) any Exchange Act Person becomes the Owner, directly or indirectly, of securities of the Company representing more than 50% of the combined voting power of the Company's then outstanding securities other than by virtue of a merger, consolidation or similar transaction. Notwithstanding the foregoing, a Change in Control will not be deemed to occur (A) on account of the acquisition of securities of the Company directly from the Company, (B) on account of the acquisition of securities of the Company by an investor, any affiliate thereof or any other Exchange Act Person that acquires the Company's securities in a transaction or series of related transactions the primary purpose of which is to obtain financing for the Company through the issuance of equity securities, or (C) solely because the level of Ownership held by any Exchange Act Person (the "***Subject Person***") exceeds the designated percentage threshold of the outstanding voting securities as a result of a repurchase or other acquisition of voting securities by the Company reducing the number of shares outstanding, provided that if a Change in Control would occur (but for the operation of this sentence) as a result of the acquisition of voting securities by the Company, and after such share acquisition, the Subject Person becomes the Owner of any additional voting securities that, assuming the repurchase or other acquisition had not occurred, increases the percentage of the then outstanding voting securities Owned by the Subject Person over the designated percentage threshold, then a Change in Control will be deemed to occur;

(ii) there is consummated a merger, consolidation or similar transaction involving (directly or indirectly) the Company and, immediately after the consummation of such merger, consolidation or similar transaction, the stockholders of the Company immediately prior thereto do not Own, directly or indirectly, either (A) outstanding voting securities representing more than 50% of the combined outstanding voting power of the surviving Entity in such merger, consolidation or similar transaction or (B) more than 50% of the combined outstanding voting power of the parent of the surviving Entity in such merger, consolidation or similar transaction, in each case in substantially the same proportions as their Ownership of the outstanding voting securities of the Company immediately prior to such transaction; *provided, however*, that a merger, consolidation or similar transaction will not constitute a Change in Control under this prong of the definition if the outstanding voting securities representing more than 50% of the combined voting power of the surviving Entity or its parent are owned by the IPO Entities;

(iii) there is consummated a sale, lease, exclusive license or other disposition of all or substantially all of the consolidated assets of the Company and its Subsidiaries, other than a sale, lease, license or other disposition of all or substantially all of the consolidated assets of the Company and its Subsidiaries to an Entity, more than 50% of the combined voting power of the voting securities of which are Owned by stockholders of the Company in substantially the same proportions as their Ownership of the outstanding voting securities of the Company immediately prior to such sale, lease, license or other disposition; *provided, however*, that a sale, lease, exclusive license or other disposition of all or substantially all of the consolidated assets of the Company and its Subsidiaries will not constitute a Change in Control under this prong of the definition if the outstanding voting securities representing more than 50% of the combined voting power of the acquiring Entity or its parent are owned by the IPO Entities; or

(iv) individuals who, on the date the Plan is adopted by the Board, are members of the Board (the "***Incumbent Board***") cease for any reason to constitute at least a majority of the members of the Board; *provided, however*, that if the appointment or election (or nomination for election) of any new Board member was approved or recommended by a majority vote of the members of the Incumbent Board then still in office, such new member will, for purposes of this Plan, be considered as a member of the Incumbent Board.

Notwithstanding the foregoing definition or any other provision of this Plan, the term Change in Control will not include a sale of assets, merger or other transaction effected exclusively for the purpose of changing the domicile of the Company and the definition of Change in Control (or any analogous term) in an individual written agreement between the Company or any Affiliate and the Participant will supersede the foregoing definition with respect to Awards subject to such agreement; *provided, however*, that if no definition of Change in Control or any analogous term is set forth in such an individual written agreement, the foregoing definition will apply.

(j) "***Code***" means the Internal Revenue Code of 1986, as amended, including any applicable regulations and guidance thereunder.

(k) "***Committee***" means a committee of one or more Directors to whom authority has been delegated by the Board in accordance with Section 2(c).

(l) "***Common Stock***" means, the common stock of the Company.

(m) "***Company***" means Zevra Therapeutics, Inc., a Delaware corporation.

(n) "***Consultant***" means any person, including an advisor, who is (i) engaged by the Company or an Affiliate to render consulting or advisory services and is compensated for such services, or (ii) serving as a member of the board of directors of an Affiliate and is compensated for such services. However, service solely as a Director, or payment of a fee for such service, will not cause a Director to be considered a "Consultant" for purposes of the Plan. Notwithstanding the foregoing, a person is treated as a Consultant under this Plan only if a Form S-8 Registration Statement under the Securities Act is available to register either the offer or the sale of the Company's securities to such person.

(o) "***Continuous Service***" means that the Participant's service with the Company or an Affiliate, whether as an Employee, Director or Consultant, is not interrupted or terminated. A change in the capacity in which the Participant renders service to the Company or an Affiliate as an Employee, Consultant or Director or a change in the entity for which the Participant renders such service, provided that there is no interruption or termination of the Participant's service with the Company or an Affiliate, will not terminate a Participant's Continuous Service; *provided, however*, that if the Entity for which a Participant is rendering services ceases to qualify as an Affiliate, as determined by the Board, in its sole discretion, such Participant's Continuous Service will be considered to have terminated on the date such Entity ceases to qualify as an Affiliate. To the extent permitted by law, the Board or the chief executive officer of the Company, in that party's sole discretion, may determine whether Continuous Service will be considered interrupted in the case of (i) any leave of absence approved by the Board or chief executive officer, including sick leave, military leave or any other personal leave, or (ii) transfers between the Company, an Affiliate, or their successors. Notwithstanding the foregoing, a leave of absence will be treated as Continuous Service for purposes of vesting in an Award only to such extent as may be provided in the Company's leave of absence policy, in the written terms of any leave of absence agreement or policy applicable to the Participant, or as otherwise required by law.

(p) "***Corporate Transaction***" means the consummation, in a single transaction or in a series of related transactions, of any one or more of the following events:

(i) a sale or other disposition of all or substantially all, as determined by the Board, in its sole discretion, of the consolidated assets of the Company and its Subsidiaries;

(ii) a sale or other disposition of at least 50% of the outstanding securities of the Company;

(iii) a merger, consolidation or similar transaction following which the Company is not the surviving corporation; or

(iv) a merger, consolidation or similar transaction following which the Company is the surviving corporation but the shares of Common Stock outstanding immediately preceding the merger, consolidation or similar transaction are converted or exchanged by virtue of the merger, consolidation or similar transaction into other property, whether in the form of securities, cash or otherwise.

(q) "***Director***" means a member of the Board.

(r) "***Disability***" means, with respect to a Participant, the inability of such Participant to engage in any substantial gainful activity by reason of any medically determinable physical or mental impairment that can be expected to result in death or that has lasted or can be expected to last for a continuous period of not less than 12 months, as provided in Sections 22(e)(3) and 409A(a)(2)(c)(i) of the Code, and will be determined by the Board on the basis of such medical evidence as the Board deems warranted under the circumstances.

(s) "***Eligible Individual***" means any individual who was not previously an Employee or Director hired as a new Employee or rehired as an Employee following a bona fide period of interruption of employment if such person is granted an Award as a material inducement to his or her entering into employment with the Company or a Subsidiary (within the meaning of the NASDAQ Rule 5635(c)(4)).

(t) "***Employee***" means any person employed by the Company or an Affiliate. However, service solely as a Director, or payment of a fee for such services, will not cause a Director to be considered an "Employee" for purposes of the Plan.

(u) "***Entity***" means a corporation, partnership, limited liability company or other entity.

(v) "***Exchange Act***" means the Securities Exchange Act of 1934, as amended, and the rules and regulations promulgated thereunder.

(w) "***Exchange Act Person***" means any natural person, Entity or "group" (within the meaning of Section 13(d) or 14(d) of the Exchange Act), except that "Exchange Act Person" will not include (i) the Company or any Subsidiary of the Company, (ii) any employee benefit plan of the Company or any Subsidiary of the Company or any trustee or other fiduciary holding securities under an employee benefit plan of the Company or any Subsidiary of the Company, (iii) an underwriter temporarily holding securities pursuant to a registered public offering of such securities, (iv) an Entity Owned, directly or indirectly, by the stockholders of the Company in substantially the same proportions as their Ownership of stock of the Company; or (v) any natural person, Entity or "group" (within the meaning of Section 13(d) or 14(d) of the Exchange Act) that, as of the Amendment Date, is the Owner, directly or indirectly, of securities of the Company representing more than 50% of the combined voting power of the Company's then outstanding securities.

(x) "***Fair Market Value***" means, as of any date, the value of the Common Stock determined as follows:

(i) If the Common Stock is listed on any established stock exchange or traded on any established market, the Fair Market Value of a share of Common Stock will be, unless otherwise determined by the Board, the closing sales price for such stock as quoted on such exchange or market (or the exchange or market with the greatest volume of trading in the Common Stock) on the date of determination, as reported in a source the Board deems reliable.

(ii) Unless otherwise provided by the Board, if there is no closing sales price for the Common Stock on the date of determination, then the Fair Market Value will be the closing selling price on the last preceding date for which such quotation exists.

(iii) In the absence of such markets for the Common Stock, the Fair Market Value will be determined by the Board in good faith and in a manner that complies with Sections 409A and 422 of the Code.

(y) "***Incentive Stock Option***" means an option intended to qualify as an "incentive stock option" within the meaning of Section 422 of the Code.

(z) "***Independent Director***" means a Director who qualifies as "independent" within the meaning of NASDAQ Rule 5635(c)(4), or any successor rule, as such rule may be amended from time to time.

(aa) "***NASDAQ Rule 5635(c)(4)***" means NASDAQ Rule 5635(c)(4), or any successor rule, and all guidance and other interpretative authority thereunder, as such rule, guidance and other authority may be amended from time to time.

(bb) "***Non-Employee Director***" means a Director who either (i) is not a current employee or officer of the Company or an Affiliate, does not receive compensation, either directly or indirectly, from the Company or an Affiliate for services rendered as a consultant or in any capacity other than as a Director (except for an amount as to which disclosure would not be required under Item 404(a) of Regulation S-K promulgated pursuant to the Securities Act ("***Regulation S-K***")), does not possess an interest in any other transaction for which disclosure would be required under Item 404(a) of Regulation S-K, and is not engaged in a business relationship for which disclosure would be required pursuant to Item 404(b) of Regulation S-K; or (ii) is otherwise considered a "non-employee director" for purposes of Rule 16b-3.

(cc) "***Nonstatutory Stock Option***" means any Option granted pursuant to Section 5 of the Plan that does not qualify as an Incentive Stock Option.

(dd) "***Officer***" means a person who is an officer of the Company within the meaning of Section 16 of the Exchange Act.

(ee) "***Option***" means a Nonstatutory Stock Option to purchase shares of Common Stock granted pursuant to the Plan.

(ff) "***Option Agreement***" means a written agreement between the Company and an Optionholder evidencing the terms and conditions of an Option grant. Each Option Agreement will be subject to the terms and conditions of the Plan.

(gg) "***Optionholder***" means a person to whom an Option is granted pursuant to the Plan or, if applicable, such other person who holds an outstanding Option.

(hh) "***Other Stock Award***" means an award based in whole or in part by reference to the Common Stock which is granted pursuant to the terms and conditions of Section 6(d).

(ii) "***Other Stock Award Agreement***" means a written agreement between the Company and a holder of an Other Stock Award evidencing the terms and conditions of an Other Stock Award grant. Each Other Stock Award Agreement will be subject to the terms and conditions of the Plan.

(jj) "***Own,***" "***Owned,***" "***Owner,***" "***Ownership***" means a person or Entity will be deemed to "Own," to have "Owned," to be the "Owner" of, or to have acquired "Ownership" of securities if such person or Entity, directly or indirectly, through any contract, arrangement, understanding, relationship or otherwise, has or shares voting power, which includes the power to vote or to direct the voting, with respect to such securities.

(kk) "***Participant***" means an Eligible Individual to whom an Award is granted pursuant to the Plan or, if applicable, such other person who holds an outstanding Stock Award.

(ll) "***Performance Cash Award***" means an award of cash granted pursuant to the terms and conditions of Section 6(c)(ii).

(mm) "***Performance Criteria***" means the one or more criteria that the Board will select for purposes of establishing the Performance Goals for a Performance Period. The Performance Criteria that will be used to establish such Performance Goals may be based on any one of, or combination of, the following as determined by the Board: (i) earnings (including earnings per share and net earnings); (ii) earnings before interest, taxes and depreciation; (iii) earnings before interest, taxes, depreciation and amortization; (iv) earnings before interest, taxes, depreciation, amortization and legal settlements; (v) earnings before interest, taxes, depreciation, amortization, legal settlements and other income (expense); (vi) earnings before interest, taxes, depreciation, amortization, legal settlements, other income (expense) and stock-based compensation; (vii) earnings before interest, taxes, depreciation, amortization, legal settlements, other income (expense), stock-based compensation and changes in deferred revenue; (viii) total stockholder return; (ix) return on equity or average stockholder's equity; (x) return on assets, investment, or capital employed; (xi) stock price; (xii) margin (including gross margin); (xiii) income (before or after taxes); (xiv) operating income; (xv) operating income after taxes; (xvi) pre-tax profit; (xvii) operating cash flow; (xviii) sales or revenue targets; (xix) increases in revenue or product revenue; (xx) expenses and cost reduction goals; (xxi) improvement in or attainment of working capital levels; (xxii) economic value added (or an equivalent metric); (xxiii) market share; (xxiv) cash flow; (xxv) cash flow per share; (xxvi) share price performance; (xxvii) debt reduction; (xxviii) implementation or completion of projects or processes; (xxix) customer satisfaction; (xxx) stockholders' equity; (xxxi) capital expenditures; (xxxii) debt levels; (xxxiii) operating profit or net operating profit; (xxxiv) workforce diversity; (xxxv) growth of net income or operating income; (xxxvi) billings; (xxxvii) bookings; (xxxviii) the number of customers, including but not limited to customers users; (xxxix) employee retention; (xl) pre-clinical development related compound goals; (xli) financing; (xlii) regulatory milestones, including approval of a compound; (xliii) stockholder liquidity; (xliv) corporate governance and compliance; (xlv) product commercialization; (xlvi) intellectual property; (xlvii) personnel matters; (xlviii) progress of internal research or clinical programs; (xlix) progress of partnered programs; (l) implementation or completion of projects and processes; (li) partner satisfaction; (lii) budget management; (liii) clinical achievements; (liv) completing phases of a clinical study (including the treatment phase); (lv) announcing or presenting preliminary or final data from clinical studies; in each case, whether on particular timelines or generally; (lvi) timely completion of clinical trials; (lvii) submission of INDs and NDAs and other regulatory achievements; (lviii) partner or collaborator achievements; (lix) internal controls, including those related to the Sarbanes-Oxley Act of 2002; (lx) research progress, including the development of programs; (lxi) investor relations, analysts and communication; (lxii) manufacturing achievements (including obtaining particular yields from manufacturing runs and other measurable objectives related to process development activities); (lxiii) strategic partnerships or transactions (including in-licensing and out-licensing of intellectual property; (lxiv) establishing relationships with commercial entities with respect to the marketing, distribution and sale of the Company's products (including with group purchasing organizations, distributors and other vendors); (lxv) supply chain achievements (including establishing relationships with manufacturers or suppliers of active pharmaceutical ingredients and other component materials and manufacturers of the Company's products); (lxvi) co-development, co-marketing, profit sharing, joint venture or other similar arrangements; and (lxvii) and other measures of performance selected by the Board.

(nn) "***Performance Goals***" means, for a Performance Period, the one or more goals established by the Board for the Performance Period based upon the Performance Criteria. Performance Goals may be based on a Company-wide basis, with respect to one or more business units, divisions, Affiliates, or business segments, and in either absolute terms or relative to the performance of one or more comparable companies or the performance of one or more relevant indices. Unless specified otherwise by the Board (i) in the Award Agreement at the time the Award is granted or (ii) in such other document setting forth the Performance Goals at the time the Performance Goals are established, the Board will appropriately make adjustments in the method of calculating the attainment of Performance Goals for a Performance Period as follows: (1) to exclude restructuring and/or other nonrecurring charges; (2) to exclude exchange rate effects; (3) to exclude the effects of changes to generally accepted accounting principles; (4) to exclude the effects of any statutory adjustments to corporate tax rates; (5) to exclude the effects of any "extraordinary items" as determined under generally accepted accounting principles; (6) to exclude the dilutive effects of acquisitions or joint ventures; (7) to assume that any business divested by the Company achieved performance objectives at targeted levels during the balance of a Performance Period following such divestiture; (8) to exclude the effect of any change in the outstanding shares of common stock of the Company by reason of any stock dividend or split, stock repurchase, reorganization, recapitalization, merger, consolidation, spin-off, combination or exchange of shares or other similar corporate change, or any distributions to common stockholders other than regular cash dividends; (9) to exclude the effects of stock based compensation and the award of bonuses under the Company's bonus plans; (10) to exclude costs incurred in connection with potential acquisitions or divestitures that are required to expensed under generally accepted accounting principles; (11) to exclude the goodwill and intangible asset impairment charges that are required to be recorded under generally accepted accounting principles, (12) to exclude the effects of the timing of acceptance for review and/or approval of submissions to the Food and Drug Administration or any other regulatory body and (13) to exclude the effect of any other unusual, non-recurring gain or loss or other extraordinary item. In addition, the Board retains the discretion to reduce or eliminate the compensation or economic benefit due upon attainment of Performance Goals and to define the manner of calculating the Performance Criteria it selects to use for such Performance Period. Partial achievement of the specified criteria may result in the payment or vesting corresponding to the degree of achievement as specified in the Stock Award Agreement or the written terms of a Performance Cash Award.

(oo) "***Performance Period***" means the period of time selected by the Board over which the attainment of one or more Performance Goals will be measured for the purpose of determining a Participant's right to and the payment of a Stock Award or a Performance Cash Award. Performance Periods may be of varying and overlapping duration, at the sole discretion of the Board.

(pp) "***Performance Stock Award***" means a Stock Award granted under the terms and conditions of Section 6(c)(i).

(qq) "***Plan***" means this 2023 Employment Inducement Award Plan, as it may be amended and/or restated from time to time.

(rr) "***Restricted Stock Award***" means an award of shares of Common Stock which is granted pursuant to the terms and conditions of Section 6(a).

(ss) "***Restricted Stock Award Agreement***" means a written agreement between the Company and a holder of a Restricted Stock Award evidencing the terms and conditions of a Restricted Stock Award grant. Each Restricted Stock Award Agreement will be subject to the terms and conditions of the Plan.

(tt) "***Restricted Stock Unit Award***" means a right to receive shares of Common Stock which is granted pursuant to the terms and conditions of Section 6(b).

(uu) "***Restricted Stock Unit Award Agreement***" means a written agreement between the Company and a holder of a Restricted Stock Unit Award evidencing the terms and conditions of a Restricted Stock Unit Award grant. Each Restricted Stock Unit Award Agreement will be subject to the terms and conditions of the Plan.

(vv) "***Rule 16b-3***" means Rule 16b-3 promulgated under the Exchange Act or any successor to Rule 16b-3, as in effect from time to time.

(ww) "***Securities Act***" means the Securities Act of 1933, as amended.

(xx) "***Stock Appreciation Right***" or "***SAR***" means a right to receive the appreciation on Common Stock that is granted pursuant to the terms and conditions of Section 5.

(yy) "***Stock Appreciation Right Agreement***" means a written agreement between the Company and a holder of a Stock Appreciation Right evidencing the terms and conditions of a Stock Appreciation Right grant. Each Stock Appreciation Right Agreement will be subject to the terms and conditions of the Plan.

(zz) "***Stock Award***" means any right to receive Common Stock granted under the Plan, including a Nonstatutory Stock Option, a Restricted Stock Award, a Restricted Stock Unit Award, a Stock Appreciation Right, a Performance Stock Award or any Other Stock Award.

(aaa) "***Stock Award Agreement***" means a written agreement between the Company and a Participant evidencing the terms and conditions of a Stock Award grant. Each Stock Award Agreement will be subject to the terms and conditions of the Plan.

(bbb) "***Subsidiary***" means, with respect to the Company, (i) any corporation of which more than 50% of the outstanding capital stock having ordinary voting power to elect a majority of the board of directors of such corporation (irrespective of whether, at the time, stock of any other class or classes of such corporation will have or might have voting power by reason of the happening of any contingency) is at the time, directly or indirectly, Owned by the Company, and (ii) any partnership, limited liability company or other entity in which the Company has a direct or indirect interest (whether in the form of voting or participation in profits or capital contribution) of more than 50%.

Exhibit 10.31

FIRST AMENDMENT TO BRIDGE LOAN AGREEMENT

This First Amendment to Bridge Loan Agreement (this "Amendment"), dated as of October 31, 2023, is by and between ACER THERAPEUTICS INC., a Delaware corporation (the "Borrower"), and ZEVRA THERAPEUTICS, INC., a Delaware corporation (the "Lender").

RECITALS

A. The Borrower and the Lender have entered into that certain Bridge Loan Agreement, dated effective August 30, 2023 (including any modifications or amendments thereto entered into from time to time prior to the date hereof, the "Loan Agreement"). Defined terms used herein and not defined herein shall have the meanings set forth in the Loan Agreement.

B. The Borrower has requested, and the Lender has agreed, subject to the terms and conditions provided in this Amendment, to amend certain terms and conditions of the Loan Agreement.

AGREEMENT

NOW THEREFORE, in consideration of the covenants, conditions and agreement hereinafter set forth, and for other good and valuable consideration, the receipt and sufficiency of which are hereby acknowledged, the Borrower and the Lender agree as follows:

1. Amendment to Loan Agreement. Subject to the conditions set forth below, including without limitation the conditions set forth in Section 2 below, and in reliance on the representations and warranties of the Borrower set forth in Section 3 below, the Loan Agreement is hereby amended by amending and restating Section 1.1(d) of the Loan Agreement in its entirety as follows:

"(d) "**Commitment**" means $18,000,000.00.".

2. Conditions Precedent. The amendment set forth in Section 1 above shall be effective as of the date of this Amendment, but only after each of the following conditions has been satisfied, in the reasonable judgment of the Lender:

a. The Borrower and the Lender shall have executed and delivered this Amendment.

b. As of the date of this Amendment, each of the representations and warranties set forth below shall be true and correct, and no default or Event of Default shall have occurred or shall result from the transactions contemplated hereby.

c. The Borrower shall have paid all fees and expenses required to be paid by the Borrower under Section 4 below.

3. Representations and Warranties. The Borrower hereby certifies to the Lender that as of the date of this Amendment, all of the Borrower's representations and warranties contained in the Loan Agreement and the Security Agreement are correct in all material respects, (i) except to the extent that any such representation and warranty refers to an earlier date, in which case such representation and warranty is true and correct in all material respects as of such earlier date and (ii) except that any such representation and warranty that is qualified as to "materiality" or similar language is true and correct (after giving effect to such qualification therein) in all respects as of the applicable date above, and no default or Event of Default has occurred. Without limiting the generality of the foregoing, the Borrower represents, warrants and agrees, as applicable, that:

a. the execution and delivery of this Amendment has been authorized by all necessary action on the part of the Borrower;

b. the person executing this Amendment on behalf of the Borrower is duly authorized to do so;

c. neither the execution, delivery nor performance of this Amendment will contravene the Borrower's organizational documents or, in any material respect, any law binding on or affecting the Borrower; and

d. this Amendment constitutes the legal, valid and binding obligation of the Borrower, enforceable against the Borrower in accordance with its terms, except as may be limited by bankruptcy laws, creditors' rights generally and general principles of equity.

4. Fees and Expenses. The Borrower shall pay to the Lender all of the reasonable and documented out of pocket expenses incurred by the Lender in connection with the transactions contemplated by this Amendment, including, without limitation, the reasonable fees and disbursements of the Lender's attorneys and their staff, and any recording, filing, lien search-related, or title-related fees, charges and expenses.

5. Additional Documents. The Borrower shall execute and deliver, and shall cause to be executed and delivered, to the Lender at any time and from time to time such documents and instruments as the Lender may reasonably request to confirm and carry out the transactions contemplated hereby.

6. Continuation of the Loan Agreement and Loan Documents. Except as specified in this Amendment, the provisions of the Loan Agreement, the Security Agreement and the Subordination Agreement shall remain in full force and effect, and if there is a conflict between the terms of this Amendment and those of the Loan Agreement or the other loan documents, the terms of this Amendment shall control.

7. Ratification and Reaffirmation of the Borrower's Obligations. Subject to the terms of this Amendment, the Borrower hereby (a) ratifies and confirms all of the Borrower's Obligations, and acknowledges and agrees that such Borrower's Obligations remain in full force and effect, and (b) ratifies, reaffirms and reapproves in favor of the Lender the terms and provisions of the Loan Agreement and each of the other loan documents, including (without limitation), its pledges and other grants of Liens and security interests pursuant to the Security Agreement.

8. Other Agreements.

a. The Borrower and the Lender agree that the Security Agreement and the Subordination Agreement are hereby amended to reflect the amendments set forth herein and that no further amendments to any loan documents are required to reflect the foregoing.

b. All references in any document to "Loan Agreement" shall refer to the Loan Agreement as amended pursuant to this Amendment.

9. Miscellaneous.

a. THIS AMENDMENT SHALL BE GOVERNED BY, AND IS TO BE CONSTRUED AND INTERPRETED IN ACCORDANCE WITH, THE LAWS OF THE STATE OF NEW YORK, WITHOUT GIVING EFFECT TO ANY CONFLICT OF LAW PRINCIPLES THAT WOULD CAUSE THE APPLICATION OF LAWS OF ANY OTHER JURISDICTION, BUT WITHOUT PREJUDICE TO OR LIMITATION OF ANY OTHER RIGHTS OR REMEDIES AVAILABLE UNDER THE LAWS OF ANY JURISDICTION WHERE PROPERTY OR ASSETS OF THE BORROWER MAY BE FOUND. This Amendment shall be binding upon and inure to the benefit of the parties hereto and their successors and permissible assigns.

b. This Amendment and all documents to be executed and delivered hereunder may be delivered in the form of a facsimile copy (or other electronic means), subsequently confirmed by delivery of the originally executed document. This Amendment may be executed in two or more counterparts, each of which shall be deemed an original and all of which together shall constitute one instrument.

c. This Amendment, the Loan Agreement, the Security Agreement, the Subordination Agreement, and all other instruments, documents and agreements executed and delivered in connection with this Amendment, the Loan Agreement, the Security Agreement and the Subordination Agreement, embody the final, entire agreement among the parties hereto with respect to the subject matter hereof. There are no oral agreements among the parties hereto. This Amendment may not be amended or modified orally, but only by a written agreement meeting the requirements of the Loan Agreement.

d. The section headings herein are for convenience only and shall not affect the construction hereof.

e. In case any provision of or obligation under this Amendment shall held by any court of competent jurisdiction to be invalid, illegal or unenforceable in any jurisdiction, the validity, legality and enforceability of the remaining provisions or obligations, or of such provision or obligation in any other jurisdiction, shall not in any way be affected or impaired thereby.

[Signature Page Follows]

IN WITNESS WHEREOF, the parties hereto have caused this Amendment to be duly executed as of the day and year first above written.

BORROWER:

ACER THERAPEUTICS INC.

By: /s/ Chris Schelling

Print Name: Chris Schelling
Title: CEO/Founder

Acer legal review: _____ CFO: _____

LENDER:

ZEVRA THERAPEUTICS, INC.

By: /s/ R. LaDuane Clifton, MBA, CPA
Print Name: R. LaDuane Clifton, MBA, CPA

Title: Chief Financial Officer, Secretary and Treasurer

Exhibit 21.1

Subsidiaries of Zevra Therapeutics, Inc.

The following companies are direct or indirect wholly owned subsidiaries of Zevra Therapeutics, Inc.:

Name	**Jurisdiction**
Acer Therapeutics Inc.	United States

Exhibit 23.1

Consent of Independent Registered Public Accounting Firm

We consent to the incorporation by reference in the following Registration Statements:

1. Registration Statements (Form S-3 Nos. 333-276856, 333-252078, 333-252903, 333-257433, 333-257661 and Form S-1 No. 333-250945) of Zevra Therapeutics, Inc.,

2. Registration Statements (Form S-8 Nos. 333-203703) pertaining to the Incentive Stock Plan, as amended, and the 2014 Equity Incentive Plan of Zevra Therapeutics, Inc.,

3. Registration Statements (Form S-8 Nos. 333-210369, 333-216858, 333-224062, 333-230041, 333-236794, 333-252743, and 333-270340) pertaining to the 2014 Equity Incentive Plan of Zevra Therapeutics, Inc., and

4. Registration Statements (Form S-8 Nos. 333-257429 and 333-270340) pertaining to the Amended and Restated 2014 Equity Incentive Plan, and the 2021 Employee Stock Purchase Plan of Zevra Therapeutics, Inc.;

5. Registration Statement (Form S-8 No. 333-270341) pertaining to the 2023 Employment Inducement Award Plan of Zevra Therapeutics, Inc.;

of our report dated April 1, 2024, with respect to the financial statements of Zevra Therapeutics, Inc. included in this Annual Report (Form 10-K) of Zevra Therapeutics, Inc. for the years ended December 31, 2023, and 2022.

/s/ Ernst & Young LLP

Orlando, Florida

April 1, 2024

Exhibit 31.1

CERTIFICATIONS

I, Neil F. McFarlane, certify that:

1. I have reviewed this Annual Report on Form 10-K of Zevra Therapeutics, Inc.;
2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;
3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;
4. The registrant's other certifying officer and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:
   (a) Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;
   (b) Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;
   (c) Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and
   (d) Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and
5. The registrant's other certifying officer and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):
   (a) All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and
   (b) Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

April 1, 2024

/s/ Neil F. McFarlane
Name: Neil F. McFarlane
Title: President and Chief Executive Officer
(Principal Executive Officer)

Exhibit 31.2

CERTIFICATIONS

I, R. LaDuane Clifton, certify that:

1. I have reviewed this Annual Report on Form 10-K of Zevra Therapeutics, Inc.;
2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;
3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;
4. The registrant's other certifying officer and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:
   (a) Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;
   (b) Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;
   (c) Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and
   (d) Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and
5. The registrant's other certifying officer and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):
   (a) All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and
   (b) Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

April 1, 2024

/s/ R. LaDuane Clifton

Name: R. LaDuane Clifton, MBA, CPA
Title: Chief Financial Officer, Secretary and Treasurer
(Principal Financial Officer)

Exhibit 32.1

CERTIFICATION OF THE PRINCIPAL EXECUTIVE OFFICER
PURSUANT TO 18 U.S.C. SECTION 1350, AS ADOPTED PURSUANT TO
SECTION 906 OF THE SARBANES-OXLEY ACT OF 2002

In connection with the Annual Report on Form 10-K of Zevra Therapeutics, Inc., (the "Company") for the fiscal year ended December 31, 2023, as filed with the Securities and Exchange Commission on the date hereof (the "Report"), I, Neil F. McFarlane, Principal Executive Officer of the Company, certify, pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, that, to my knowledge:

1. The Report fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934; and
2. The information contained in the Report fairly presents, in all material respects, the financial condition and results of operations of the Company.

April 1, 2024

/s/ Neil F. McFarlane
Name: Neil F. McFarlane
Title: President and Chief Executive Officer
(Principal Executive Officer)

The foregoing certification is being furnished solely pursuant to 18 U.S.C. Section 1350, is not being "filed" by the Company as part of the Report or as a separate disclosure document and is not to be incorporated by reference into any filing of the Company under the Securities Act of 1933, as amended, or the Securities Exchange Act of 1934, as amended (whether made before or after the date of the Report), irrespective of any general incorporation language contained in such filing.

Exhibit 32.2

CERTIFICATION OF THE PRINCIPAL FINANCIAL OFFICER
PURSUANT TO 18 U.S.C. SECTION 1350, AS ADOPTED PURSUANT TO
SECTION 906 OF THE SARBANES-OXLEY ACT OF 2002

In connection with the Annual Report on Form 10-K of Zevra Therapeutics, Inc., (the "Company") for the fiscal year ended December 31, 2023, as filed with the Securities and Exchange Commission on the date hereof (the "Report"), I, R. LaDuane Clifton, Principal Financial Officer of the Company, certify, pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, that, to my knowledge:

1. The Report fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934; and
2. The information contained in the Report fairly presents, in all material respects, the financial condition and results of operations of the Company.

April 1, 2024

/s/ R. LaDuane Clifton
Name: R. LaDuane Clifton, MBA, CPA
Title: Chief Financial Officer, Secretary and Treasurer
(Principal Financial Officer)

The foregoing certification is being furnished solely pursuant to 18 U.S.C. Section 1350, is not being "filed" by the Company as part of the Report or as a separate disclosure document and is not to be incorporated by reference into any filing of the Company under the Securities Act of 1933, as amended, or the Securities Exchange Act of 1934, as amended (whether made before or after the date of the Report), irrespective of any general incorporation language contained in such filing.

ZEVRA THERAPEUTICS, INC.

POLICY FOR RECOVERY OF ERRONEOUSLY AWARDED COMPENSATION

Zevra Therapeutics, Inc. (the "***Company***") has adopted this Policy for Recovery of Erroneously Awarded Compensation (the "***Policy***"), effective as of October 2, 2023 (the "***Effective Date***"). Capitalized terms used in this Policy but not otherwise defined herein are defined in Section 11.

1. Persons Subject to Policy

This Policy shall apply to current and former Officers of the Company. Each Officer shall be required to sign an acknowledgment pursuant to which such Officer will agree to be bound by the terms of, and comply with, this Policy; however, any Officer's failure to sign any such acknowledgment shall not negate the application of this Policy to the Officer.

2. Compensation Subject to Policy

This Policy shall apply to Incentive-Based Compensation received on or after the Effective Date. For purposes of this Policy, the date on which Incentive-Based Compensation is "received" shall be determined under the Applicable Rules, which generally provide that Incentive-Based Compensation is "received" in the Company's fiscal period during which the relevant Financial Reporting Measure is attained or satisfied, without regard to whether the grant, vesting or payment of the Incentive-Based Compensation occurs after the end of that period.

3. Recovery of Compensation

In the event that the Company is required to prepare a Restatement, the Company shall recover, reasonably promptly and in accordance with Section 4 below, the portion of any Incentive-Based Compensation that is Erroneously Awarded Compensation, unless the Committee has determined that recovery would be Impracticable. Recovery shall be required in accordance with the preceding sentence regardless of whether the applicable Officer engaged in misconduct or otherwise caused or contributed to the requirement for the Restatement and regardless of whether or when restated financial statements are filed by the Company. For clarity, the recovery of Erroneously Awarded Compensation under this Policy will not give rise to any person's right to voluntarily terminate employment for "good reason," or due to a "constructive termination" (or any similar term of like effect) under any plan, program or policy of or agreement with the Company or any of its affiliates.

4. Manner of Recovery; Limitation on Duplicative Recovery

The Committee shall, in its sole discretion, determine the manner of recovery of any Erroneously Awarded Compensation, which may include, without limitation, reduction or cancellation by the Company or an affiliate of the Company of Incentive-Based Compensation or Erroneously Awarded Compensation, reimbursement or repayment by any person subject to this Policy, and, to the extent permitted by law, an offset of the Erroneously Awarded Compensation against other compensation payable by the Company or an affiliate of the Company to such person.

Notwithstanding the foregoing, unless otherwise prohibited by the Applicable Rules, to the extent this Policy provides for recovery of Erroneously Awarded Compensation already recovered by the Company pursuant to Section 304 of the Sarbanes-Oxley Act of 2002 or Other Recovery Arrangements, the amount of Erroneously Awarded Compensation already recovered by the Company from the recipient of such Erroneously Awarded Compensation may be credited to the amount of Erroneously Awarded Compensation required to be recovered pursuant to this Policy from such person.

5. Administration

This Policy shall be administered, interpreted and construed by the Committee, which is authorized to make all determinations necessary, appropriate or advisable for such purpose. The Board of Directors of the Company (the "***Board***") may re-vest in itself the authority to administer, interpret and construe this Policy in accordance with applicable law, and in such event references herein to the "Committee" shall be deemed to be references to the Board. Subject to any permitted review by the applicable national securities exchange or association pursuant to the Applicable Rules, all determinations and decisions made by the Committee pursuant to the provisions of this Policy shall be final, conclusive and binding on all persons, including the Company and its affiliates, equityholders and employees. The Committee may delegate administrative duties with respect to this Policy to one or more directors or employees of the Company, as permitted under applicable law, including any Applicable Rules.

6. Interpretation

This Policy will be interpreted and applied in a manner that is consistent with the requirements of the Applicable Rules, and to the extent this Policy is inconsistent with such Applicable Rules, it shall be deemed amended to the minimum extent necessary to ensure compliance therewith.

7. No Indemnification; No Liability

The Company shall not indemnify or insure any person against the loss of any Erroneously Awarded Compensation pursuant to this Policy, nor shall the Company directly or indirectly pay or reimburse any person for any premiums for third-party insurance policies that such person may elect to purchase to fund such person's potential obligations under this Policy. None of the Company, an affiliate of the Company or any member of the Committee or the Board shall have any liability to any person as a result of actions taken under this Policy.

8. Application; Enforceability

Except as otherwise determined by the Committee or the Board, the adoption of this Policy does not limit, and is intended to apply in addition to, any other clawback, recoupment, forfeiture or similar policies or provisions of the Company or its affiliates, including any such policies or provisions of such effect contained in any employment agreement, bonus plan, incentive plan, equity-based plan or award agreement thereunder or similar plan, program or agreement of the Company or an affiliate or required under applicable law (the "***Other Recovery Arrangements***"). The remedy specified in this Policy shall not be exclusive and shall be in addition to every other right or remedy at law or in equity that may be available to the Company or an affiliate of the Company or is otherwise required by applicable law and regulations.

9. Severability

The provisions in this Policy are intended to be applied to the fullest extent of the law; provided, however, to the extent that any provision of this Policy is found to be unenforceable or invalid under any applicable law, such provision will be applied to the maximum extent permitted, and shall automatically be deemed amended in a manner consistent with its objectives to the extent necessary to conform to any limitations required under applicable law.

10. Amendment and Termination

The Board or the Committee may amend, modify or terminate this Policy in whole or in part at any time and from time to time in its sole discretion. This Policy will terminate automatically when the Company does not have a class of securities listed on a national securities exchange or association.

11. Definitions

"*Applicable Rules*" means Section 10D of the Exchange Act, Rule 10D-1 promulgated thereunder, the listing rules of the national securities exchange or association on which the Company's securities are listed, and any applicable rules, standards or other guidance adopted by the Securities and Exchange Commission or any national securities exchange or association on which the Company's securities are listed.

"*Committee*" means the Compensation Committee of the Board, provided that, for purposes of determining whether recovery of Incentive-Based Compensation that is Erroneously Awarded Compensation would be Impracticable, "***Committee***" shall mean the committee of the Board responsible for executive compensation decisions comprised solely of independent directors (as determined under the Applicable Rules), or in the absence of such a committee, a majority of the independent directors serving on the Board.

"*Erroneously Awarded Compensation*" means the amount of Incentive-Based Compensation received by a current or former Officer that exceeds the amount of Incentive-Based Compensation that would have been received by such current or former Officer based on a restated Financial Reporting Measure, as determined on a pre-tax basis in accordance with the Applicable Rules.

"*Exchange Act*" means the Securities Exchange Act of 1934, as amended.

"*Financial Reporting Measure*" means any measure determined and presented in accordance with the accounting principles used in preparing the Company's financial statements, and any measures derived wholly or in part from such measures, including GAAP, IFRS and non- GAAP/IFRS financial measures, as well as stock or share price and total equityholder return.

"*GAAP*" means United States generally accepted accounting principles.

"*IFRS*" means international financial reporting standards as adopted by the International Accounting Standards Board.

"*Impracticable*" means (a) the direct expenses paid to third parties to assist in enforcing recovery would exceed the Erroneously Awarded Compensation; provided that the Company has (i) made reasonable attempts to recover the Erroneously Awarded Compensation, (ii) documented such attempt(s), and (iii) provided such documentation to the relevant listing exchange or association, (b) to the extent permitted by the Applicable Rules, the recovery would violate the Company's home country laws pursuant to an opinion of home country counsel; provided that the Company has (i) obtained an opinion of home country counsel, acceptable to the relevant listing exchange or association, that recovery would result in such a violation, and (ii) provided such opinion to the relevant listing exchange or association, or (c) recovery would likely cause an otherwise tax-qualified retirement plan, under which benefits are broadly available to employees of the Company, to fail to meet the requirements of 26 U.S.C. 401(a)(13) or 26 U.S.C. 411(a) and the regulations thereunder.

"*Incentive-Based Compensation*" means, with respect to a Restatement, any compensation that is granted, earned, or vested based wholly or in part upon the attainment of one or more Financial Reporting Measures and received by a person: (a) after such person began service as an Officer; (b) who served as an Officer at any time during the performance period for that compensation; (c) while the Company has a class of its securities listed on a national securities exchange or association; and (d) during the applicable Three-Year Period.

"*Officer*" means each person who serves as an executive officer of the Company, as defined in Rule 10D-1(d) under the Exchange Act.

"*Restatement*" means an accounting restatement to correct the Company's material noncompliance with any financial reporting requirement under securities laws, including restatements that correct an error in previously issued financial statements (a) that is material to the previously issued financial statements or (b) that would result in a material misstatement if the error were corrected in the current period or left uncorrected in the current period.

"*Three-Year Period*" means, with respect to a Restatement, the three completed fiscal years immediately preceding the date that the Board, a committee of the Board, or the officer or officers of the Company authorized to take such action if Board action is not required, concludes, or reasonably should have concluded, that the Company is required to prepare such Restatement, or, if earlier, the date on which a court, regulator or other legally authorized body directs the Company to prepare such Restatement. The "Three-Year Period" also includes any transition period (that results from a change in the Company's fiscal year) within or immediately following the three completed fiscal years identified in the preceding sentence. However, a transition period between the last day of the Company's previous fiscal year end and the first day of its new fiscal year that comprises a period of nine to 12 months shall be deemed a completed fiscal year.

ACKNOWLEDGMENT AND CONSENT TO
POLICY FOR RECOVERY OF ERRONEOUSLY AWARDED COMPENSATION

The undersigned has received a copy of the Policy for Recovery of Erroneously Awarded Compensation (the "***Policy***") adopted by Zevra Therapeutics, Inc. (the "***Company***").

For good and valuable consideration, the receipt of which is acknowledged, the undersigned agrees to the terms of the Policy and agrees that compensation received by the undersigned may be subject to reduction, cancellation, forfeiture and/or recoupment to the extent necessary to comply with the Policy, notwithstanding any other agreement to the contrary. To the extent any recovery right under the Policy and any Other Recovery Arrangements (as defined in the Policy) applicable to the undersigned conflicts with any other contractual rights the undersigned may have with the Company or any affiliate, the undersigned understands that the terms of the Policy and the Other Recovery Arrangements shall supersede any such contractual rights.

The undersigned further acknowledges and agrees that the undersigned is not entitled to indemnification in connection with any enforcement of the Policy and expressly waives any rights to such indemnification under the Company's organizational documents or otherwise.

Date

Signature

Name

Title