

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

FORM 8-K

CURRENT REPORT

Pursuant to Section 13 or 15(d) of the Securities Exchange Act of 1934

Date of Report (Date of Earliest Event Reported): May 13, 2023

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

Delaware
(State or Other Jurisdiction of Incorporation)

001-36913
(Commission File Number)

20-5894398
(IRS Employer Identification No.)

1180 Celebration Boulevard, Suite 103, Celebration, FL
(Address of Principal Executive Offices)

34747
(Zip Code)

Registrant's Telephone Number, Including Area Code: (321) 939-3416

(Former Name or Former Address, if Changed Since Last Report)

Check the appropriate box below if the Form 8-K filing is intended to simultaneously satisfy the filing obligation of the registrant under any of the following provisions (see General Instructions A.2. below):

- Written communications pursuant to Rule 425 under the Securities Act (17 CFR 230.425)
- Soliciting material pursuant to Rule 14a-12 under the Exchange Act (17 CFR 240.14a-12)
- Pre-commencement communications pursuant to Rule 14d-2(b) under the Exchange Act (17 CFR 240.14d-2(b))
- Pre-commencement communications pursuant to Rule 13e-4(c) under the Exchange Act (17 CFR 240.13e-4(c))

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

Title of each class	Trading Symbol(s)	Name of each exchange on which registered
Common Stock, par value \$0.0001 per share	ZVRA	The Nasdaq Stock Market LLC (Nasdaq Global Select Market)

Indicate by check mark whether the registrant is an emerging growth company as defined in Rule 405 of the Securities Act of 1933 (§ 230.405 of this chapter) or Rule 12b-2 of the Securities Exchange Act of 1934 (§ 240.12b-2 of this chapter).

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.

Item 2.02 Results of Operations and Financial Condition.

On May 15, 2023, Zevra Therapeutics, Inc., a Delaware corporation ("Zevra" or "the Company"), issued a press release announcing its financial results for the first quarter ended March 31, 2023, as well as information regarding a conference call and live audio webcast with slide presentation to discuss its financial results and corporate updates scheduled for Monday, May 15, 2023, at 4:30 p.m. ET.

A copy of the press release and presentation are furnished as Exhibit 99.1 and Exhibit 99.2, respectively, to this Current Report on Form 8-K. The information contained in the press release and presentation, furnished as Exhibit 99.1 and Exhibit 99.2, respectively, shall not be deemed "filed" for purposes of Section 18 of the Securities Exchange Act of 1934, as amended, and is not incorporated by reference into any of Zevra's filings under the Securities Act of 1933, as amended, whether made before or after the date hereof, except as shall be expressly set forth by specific reference in any such filing.

Item 5.02 Departure of Directors or Certain Officers; Election of Directors; Appointment of Certain Officers; Compensatory Arrangements of Certain Officers.

On May 13, 2023, Zevra's Board of Directors (the "Board") appointed Christal M.M. Mickle, Zevra's Chief Development Officer, as Zevra's interim President and Chief Executive Officer and (ii) designated Ms. Mickle as Zevra's principal executive officer, succeeding Richard W. Pascoe, Zevra's current Chief Executive Officer, in such role, in each case effective as of June 1, 2023 (the "Effective Date"). Ms. Mickle will continue to serve as Zevra's Chief Development Officer while she serves as Zevra's interim President and Chief Executive Officer. As previously reported, Richard W. Pascoe resigned as Zevra's Chief Executive Officer, effective as of the Effective Date.

Christal M.M. Mickle, age 44, has served as Zevra's Chief Development Officer since January 2023. Ms. Mickle, who co-founded and has held a variety of positions at Zevra, most recently 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 founding Zevra in 2006, Ms. Mickle started her career as a Research Associate for New River Pharmaceuticals, preparing compounds in attention-deficit/hyperactivity disorder, 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 U.S. Food and Drug Administration. In addition, her efforts managing a team of talented scientists has led to the approval of three New Drug Applications. 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.

In connection with Ms. Mickle's appointment as interim President and Chief Executive Officer, Zevra and Ms. Mickle entered into a letter agreement with Ms. Mickle pursuant to which she will be paid at an annual rate of \$512,000 while she serves as interim President and Chief Executive Officer. Ms. Mickle will continue to serve as interim President and Chief Executive Officer until a new Chief Executive Officer is appointed, or such other date that the Board determines.

The foregoing description of the Amendment does not purport to be complete and is qualified in its entirety by reference to the full text of the Amendment, a copy of which is attached as Exhibit 10.1 hereto and is incorporated herein by reference.

In connection with her appointment, Ms. Mickle has also entered into Zevra's standard indemnification agreement for directors and officers.

Item 9.01 Financial Statements and Exhibits.

(d) Exhibits

Exhibit No.	Description
10.1	Agreement Regarding Employment Terms, dated as of May 13, 2023, between Zevra Therapeutics, Inc. and Christal M.M. Mickle
99.1	Press Release dated May 15, 2023.
99.2	Presentation dated May 15, 2023.
104	Cover Page Interactive Data File (embedded within the Inline XBRL document)

SIGNATURES

Pursuant to the requirements 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.

Date: May 15, 2023

By: /s/ R. LaDuane Clifton
R. LaDuane Clifton, CPA
Chief Financial Officer, Secretary and Treasurer



May 13, 2023

Re: Agreement regarding Employment Terms

Dear Christal:

This letter confirms the agreement between you and Zevra Therapeutics, Inc., f/k/a KemPharm, Inc. (the "Company") to modify certain terms of your employment during your period of service as interim Chief Executive Officer and President of the Company. Reference is made to the Employment Agreement, dated as of May 30, 2014, by and between you and the Company, as amended (the "Employment Agreement"). Capitalized terms used but not defined in this letter have the meanings given in the Employment Agreement.

Title and Responsibilities. Effective as of June 1, 2023 (the "Transition Date") and ending on the date that a new Chief Executive Officer of the Company is appointed, or such other date that the Board of Directors determines (such term, the "Transition Term"), you agree to serve as the Company's interim Chief Executive Officer and President, with such responsibilities, duties and authority normally associated with such position. During the Transition Term, you will report to the Board of Directors and will dedicate your business time to the affairs of the Company as is reasonably necessary to perform your duties as interim Chief Executive Officer and President. For the avoidance of doubt, during the Transition Term you will also continue to serve as the Company's Chief Development Officer.

Salary and Benefits. During the Transition Term, your Base Salary will be paid at an annual rate of \$512,000 (which for the avoidance of doubt is in lieu of, and not in addition to, the Base Salary amount otherwise provided under the Employment Agreement and payable during the Transition Term), prorated for any partial period of service and less applicable withholdings, payable on a monthly or more frequent periodic basis in accordance with the normal payroll practices of the Company. The Target Annual Bonus amount will remain the same as the amount in effect immediately prior to the Transition Date. During the Transition Term, you will continue to be eligible to participate in the same employee benefit plans that you participated in on the Transition Date, subject to the terms and conditions of such plans.

Following expiration of the Transition Term, the terms of your employment shall revert to those in the Employment Agreement as in effect on the Transition Date, which you agree will not constitute Good Reason under the Employment Agreement. This letter agreement represents the entire agreement between the parties hereto regarding the subject matter hereof and supersedes any prior written or oral agreements regarding such subject matter. Except as otherwise provided in this letter, the Employment Agreement will remain in full force and effect in accordance with its terms.

[Signature Page Follows]

Please indicate your agreement to the foregoing by returning a countersigned copy of this letter to the Company. Thank you for your continued service to the Company.

Sincerely,

ZEVRA THERAPEUTICS, INC.

By: /s/ R. LaDuane Clifton

Name: R. LaDuane Clifton, CPA

Title: Chief Financial Officer, Secretary and Treasurer

ACKNOWLEDGED AND AGREED:

/s/ Christal M.M. Mickle

Christal M.M. Mickle



Zevra Therapeutics Reports Corporate Updates and First Quarter 2023 Financial Results

Christal Mickle, Co-Founder and Chief Development Officer, appointed to serve as interim President and CEO effective June 1, 2023

Conference call and live audio webcast with slide presentation scheduled for today, May 15, 2023, 4:30 p.m. ET

Celebration, FL – May 15, 2023 – Zevra Therapeutics, Inc. (NasdaqGS: ZVRA) (Zevra, or the Company, formerly KemPharm, Inc.), a rare disease therapeutics company, today provided corporate updates and reported its financial results for the quarter ended March 31, 2023.

“Zevra is moving at full speed as a company strategically focused on developing and commercializing novel therapeutics designed to address the unmet needs of people with rare diseases,” said Christal Mickle, Chief Development Officer of Zevra Therapeutics. “To this end, we continue to advance arimoclomol, our NDA-stage investigational product candidate for Niemann-Pick disease type C (NPC) as we complete our preparation of the NDA for resubmission. KP1077, our lead clinical candidate for treating idiopathic hypersomnia (IH), is advancing with plans to expand into narcolepsy.”

LaDuane Clifton, Chief Financial Officer, added, “Our pipeline development efforts are supported by a strong financial foundation with growing revenues from AZSTARYS® royalties and potential milestone payments, as well as from the arimoclomol early access program in France. With \$95.3 million in cash, cash equivalents, and investments as of March 31, 2023, our cash runway is expected to extend into 2026.”

Recent Business and Corporate Highlights:

- Rebranded Zevra Therapeutics, reinforcing the Company's strategic transformation into a commercially focused rare disease company.
 - Continued advancement of arimoclomol toward resubmission of its New Drug Application (NDA) to the U.S. Food and Drug Administration (FDA) anticipated as early as Q3 2023:
 - Safety and efficacy data from the four-year open-label extension of the Phase 2/3 clinical trial of arimoclomol in NPC were publicly announced and will be included as part of the updated arimoclomol NDA.
 - Continued advancement of Phase 2 clinical trial evaluating KP1077 as a treatment for IH:
 - Interim efficacy and safety data are expected as early as Q3 2023, with the potential for topline Phase 2 data as early as year-end 2023.
 - Data gathered from the Phase 2 trial in IH will support the advancement of KP1077 into a pivotal Phase 3 trial in patients with IH, as well as inform the decision of whether to initiate a future Phase 3 trial in patients with narcolepsy.
 - Expanded the clinical program for KP1077 by opening an IND for narcolepsy, extending its potential to address multiple rare sleep disorders.
 - Strong balance sheet, with \$95.3 million in cash, cash equivalents, and investments as of March 31, 2023, which supports our operating cash runway into 2026:
 - Announced Board and Chief Executive Officer (CEO) changes:
 - John B. Bode, Douglas E. Calder, Wendy Dixon and Corey Watton joined the Board of Directors at the 2023 Annual Meeting of Stockholders.
 - Tamara A. Favorito was unanimously appointed Chair of the Board of Directors.
 - Richard W. Pascoe resigned from his role as CEO, effective June 1, 2023.
 - Christal Mickle, Co-Founder and Chief Development Officer, appointed to serve as interim CEO and President effective June 1, 2023.
 - The Board will begin a search for both a new CEO and replacement Board members for Matthew Plooster and Joseph B. Saluri who have announced their intention to not stand for re-election at the Company's 2024 Annual Meeting of Stockholders, and to retire from the Board as soon as replacements are found.
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Overview of Q1 2023 Financial Results:

Net revenue for Q1 2023 was \$2.9 million compared to Q1 2022 net revenue of \$4.0 million. Ongoing reimbursements from the French early access program for arimoclomol, AZSTARYS® royalty revenues, and consulting fees primarily drove Q1 2023 net revenue.

Research and development (R&D) expenses were \$8.8 million for Q1 2023, compared to \$3.1 million in Q1 2022. The increase in R&D expenses were primarily driven by expanding activity for the KP1077 clinical development program and the addition of the arimoclomol program in Q2 2022.

General and administrative (G&A) expenses were \$6.8 million for Q1 2023, compared to \$2.7 million in Q1 2022. The period-over-period increase was primarily related to the arimoclomol acquisition, which led to an increase in compensation costs, as well as increased professional fees and depreciation and amortization expenses beginning in Q2 2022.

Net loss attributable to common stockholders for Q1 2023 was (\$11.8) million, or (\$0.34) per basic and diluted share, compared to a net loss attributable to common stockholders of (\$1.9) million, or (\$0.05) per basic and diluted share for the same period in 2022.

During Q1 2023, the Company repurchased 665,739 shares of the Company's common stock for approximately \$3.4 million, at an average price of \$5.09 per share. Approximately \$39.1 million remains available under the Share Repurchase Program as of March 31, 2023.

As of March 31, 2023, total cash, cash equivalents, and investments were \$95.3 million, a decrease of \$7.6 million compared to \$102.9 million as of December 31, 2022. The decrease was driven, in part, by increased third-party R&D costs related to the KP1077 clinical trial program and the arimoclomol program, increased G&A expenses, and share repurchases during the period.

Based on the Company's current operating forecast, existing cash, cash equivalents, and investments are expected to be sufficient to continue operations into 2026. The operating forecast includes the remaining activities required to resubmit the arimoclomol NDA, funding of the commercial preparation for potential launch of arimoclomol in the U.S., if approved, completion of the development program for KP1077 in IH, including preparation and submission of the NDA and up to the potential PDUFA date.

As of March 31, 2023, the total shares of common stock outstanding were 33,881,804, and the fully diluted common shares outstanding was 49,307,811, which included 4,252,490 shares issuable upon exercise of warrants.

Conference Call Information:

Zevra will host a conference call and live audio webcast with a slide presentation today at 4:30 p.m. ET, to discuss its corporate and financial results for Q1 2023.

The audio webcast with slide presentation will be accessible via the Investor Relations section of the Company's website, <http://investors.zevra.com/>. An archive of the webcast and presentation will be available for 90 days beginning at approximately 5:30 p.m. ET, on May 15, 2023.

Additionally, interested participants and investors may access conference call by dialing either:

- (800) 267-6316 (U.S.)
- +1 (203) 518-9783 (International)
- Conference ID: ZVRAQ123

About Zevra:

Zevra Therapeutics is a rare disease company melding science, data, and patient need to create transformational therapies for diseases with limited or no treatment options. With unique, data-driven clinical, regulatory, and commercialization strategies, the Company is overcoming complex drug development challenges to bring much-needed therapies to patients.

Arimoclomol, Zevra's orally-delivered, first-in-class investigational product candidate for the treatment of Niemann-Pick disease type C (NPC), has been granted orphan drug designation, Fast Track designation, Breakthrough Therapy designation and rare pediatric disease designation for NPC by the U.S. Food and Drug Administration (FDA), and orphan medicinal product designation for the treatment of NPC by the European Medicines Agency (EMA). The arimoclomol New Drug Application (NDA) is currently being prepared for a resubmission to the FDA.

KP1077 is Zevra's lead clinical candidate being developed to treat idiopathic hypersomnia (IH) and narcolepsy. KP1077 is comprised solely of serdexmethylphenidate (SDX), Zevra's proprietary prodrug of d-methylphenidate ("d-MPH"). The FDA has granted KP1077 orphan drug designation for the treatment of IH, and the U.S. Drug Enforcement Agency (DEA) has classified SDX as a Schedule IV controlled substance based on evidence suggesting SDX has a lower potential for abuse when compared to d-MPH, a Schedule II controlled substance.

Early access programs are made available by Zevra Therapeutics, Inc. and its affiliates and are subject to the Company's Early Access Program (EAP) policy as published on its website at zevra.com. Participation in these programs is subject to the laws and regulations of each jurisdiction under which each respective program is operated. Eligibility for participation in any such program is at the treating physician's discretion.

Caution Concerning Forward Looking Statements:

This press release may contain forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995. Forward-looking statements include all statements that do not relate solely to historical or current facts, and which can be identified by the use of words such as "may," "will," "expect," "project," "estimate," "anticipate," "plan," "believe," "potential," "should," "continue," "could," "intend," "target," "predict," or the negative versions of those words or other comparable words or expressions, although not all forward-looking statements contain these identifying words or expressions. Forward-looking statements are not guarantees of future actions or performance. These forward-looking statements include without limitation statements regarding senior leadership and board member transitions and refreshment, or the timing thereof, and our strategic and product development objectives, the promise and potential impact of our preclinical or clinical trial data, including without limitation the initiation, timing and results of any clinical trials or readouts, the content, timing or results of any Investigational New Drug ("IND") applications and New Drug Application ("NDA") submissions or resubmissions for arimoclomol, KP1077, or any other product candidates for any specific disease indication or at any dosage, the potential achievement of commercial sales or revenue milestones for AZSTARYS and the timing thereof, the sufficiency of our cash, cash equivalents and investments to fund our operating activities for any specific period of time, expected net revenue from the French EAP program, and our strategic and product development objectives, including with respect to becoming a leading, commercially-focused rare disease company. Forward-looking statements are based on information currently available to Zevra and its current plans or expectations. They are subject to several known and unknown uncertainties, risks, and other important factors that may cause our actual results, performance, or achievements to be materially different from any future results, performance, or achievements expressed or implied by the forward-looking statements. These and other important factors are described in detail in the "Risk Factors" section of Zevra's (formerly KemPharm) Annual Report on Form 10-K for the year ended December 31, 2022, as updated in Zevra's (formerly KemPharm) Quarterly Report on Form 10-Q for the quarter ended March 31, 2023, and Zevra's (formerly KemPharm) other filings with the Securities and Exchange Commission. While we may elect to update such forward-looking statements at some point in the future, except as required by law, we disclaim any obligation to do so, even if subsequent events cause our views to change. Although we believe the expectations reflected in such forward-looking statements are reasonable, we cannot assure that such expectations will prove correct. These forward-looking statements should not be relied upon as representing our views as of any date after the date of this press release.

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Contacts:

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

	Three months ended March 31,	
	2023	2022
Revenue, net	\$ 2,879	\$ 3,965
Operating expenses:		
Cost of revenue	125	8
Research and development	8,844	3,082
General and administrative	6,834	2,734
Total operating expenses	15,803	5,824
(Loss) income from operations	(12,924)	(1,859)
Other (expense) income:		
Interest expense	(182)	(5)
Fair value adjustment related to derivative and warrant liability	(2)	241
Fair value adjustment related to investments	196	(352)
Interest and other income, net	1,042	107
Total other income (expense)	1,054	(9)
Loss before income taxes	(11,870)	(1,868)
Income tax benefit	103	4
Net loss	\$ (11,767)	\$ (1,864)
Net loss attributable to common stockholders	\$ (11,767)	\$ (1,864)
Basic and diluted net loss per share of common stock:		
Net loss attributable to common stockholders	\$ (0.34)	\$ (0.05)
Weighted average number of shares of common stock outstanding:		
Basic and diluted	34,466,542	34,506,597

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

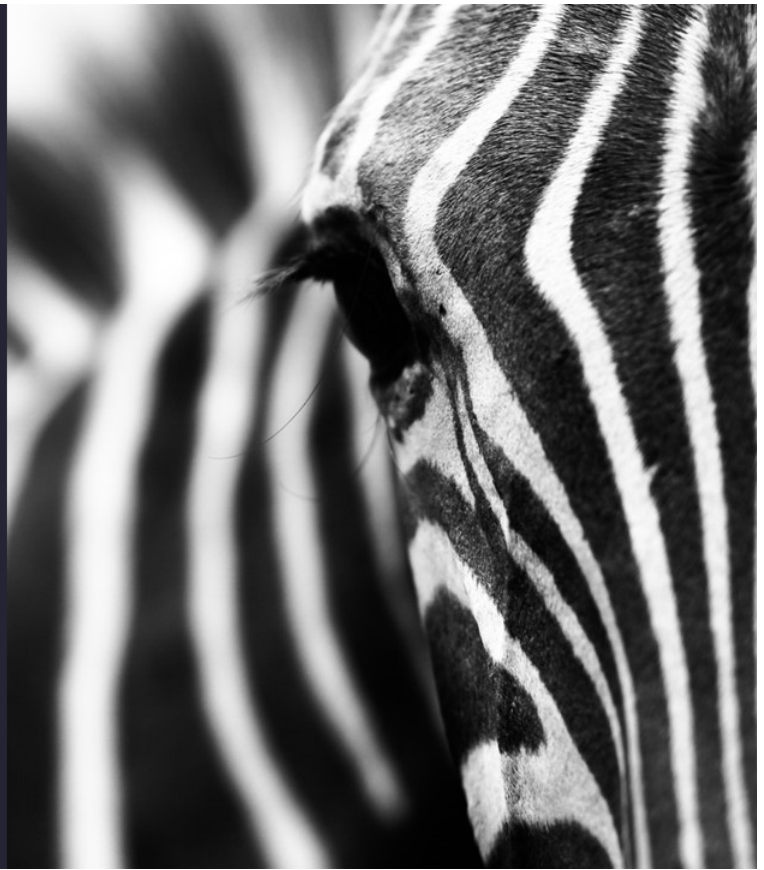
	March 31, 2023	December 31, 2022
Assets		
Current assets:		
Cash and cash equivalents	\$ 40,181	\$ 65,466
Securities at fair value	34,403	16,900
Short-term investments - other	20,700	481
Accounts and other receivables	7,822	8,299
Prepaid expenses and other current assets	1,174	1,877
Total current assets	104,280	93,023
Inventories	620	671
Property and equipment, net	744	794
Operating lease right-of-use assets	898	988
Long-term investments - other	—	20,000
Other long-term assets	53	53
Total assets	\$ 106,595	\$ 115,529
Liabilities and stockholders' equity		
Current liabilities:		
Accounts payable and accrued expenses	\$ 10,098	\$ 6,169
Current portion of operating lease liabilities	470	480
Current portion of discount and rebate liabilities	4,746	4,655
Other current liabilities	302	422
Total current liabilities	15,616	11,726
Line of credit payable	12,914	12,800
Derivative and warrant liability	3	1
Operating lease liabilities, less current portion	736	843
Discount and rebate liabilities, less current portion	5,764	4,327
Other long-term liabilities	158	25
Total liabilities	35,191	29,722
Commitments and contingencies		
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
Additional paid-in capital	402,786	401,799
Treasury stock, at cost	(10,983)	(7,536)
Accumulated deficit	(320,339)	(308,572)
Accumulated other comprehensive (loss) income	(63)	113
Total stockholders' equity	71,404	85,807
Total liabilities and stockholders' equity	\$ 106,595	\$ 115,529

Q1 2023 Results

May 15, 2023



Trademarks herein are held by their respective owners.



Cautionary Note Regarding Presentation Information

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While we may elect to update such forward-looking statements at some point in the future, except as required by law, we disclaim any obligation to do so, even if subsequent events cause our views to change. Although we believe the expectations reflected in such forward-looking statements are reasonable, we can give no assurance that such expectations will prove to be correct. These forward-looking statements should not be relied upon as representing our views as of any date subsequent to this presentation.

This presentation also may contain estimates and other statistical data made by independent parties and by us relating to market size and other data about our industry. This data involves a number of assumptions and limitations, and you are cautioned not to give undue weight to such estimates. In addition, projections, assumptions and estimates of our future performance and the future performance of the markets in which we operate are necessarily subject to a high degree of uncertainty and risk.

Agenda



First Quarter 2023 Overview

- Tamara Favorito, Zevra Board Chair

Highlights and Program Updates

- Christal Mickle, Chief Development Officer, Co-Founder, and Interim CEO and President effective June 1, 2023

AZSTARYS® Commercial Update, First Quarter 2023 Financial Results & 2023 Financial Guidance

- R. LaDuane Clifton, Chief Financial Officer

Q&A

- Tamara Favorito, Zevra Board Chair
- Christal Mickle, Chief Development Officer, Co-Founder, and Interim CEO and President effective June 1, 2023
- R. LaDuane Clifton, Chief Financial Officer
- Joshua Schafer, Chief Commercial Officer and Executive Vice President, Business Development

Changes to Leadership Team and Board

Advancing the rare disease pipeline remains the top priority

- Announced Board and Chief Executive Officer (CEO) changes:
 - John B. Bode, Douglas W. Calder, Wendy Dixon and Corey Watton joined the Board of Directors at the 2023 Annual Meeting of Stockholders;
 - Tamara A. Favorito was unanimously appointed Chair of the Board of Directors;
 - Richard W. Pascoe resigned from his role as CEO, effective June 1, 2023
 - The Board will engage in a search for both a new CEO and replacement Board members for Matthew Plooster and Joseph B. Saluri who indicated their intention to not stand for re-election at the Company's 2024 Annual Meeting of Stockholders, and to retire from the Board as soon as replacements are found
- Christal Mickle, currently our Chief Development Officer and Co-Founder, will be taking on the role of interim President and CEO effective June 1, 2023

1Q 2023 Highlights

Arimoclomol for Niemann-Pick Type C

- NDA resubmission as early as Q3 2023
- Completion of 4-year safety trial
- Data presentation at *WorldSymposium 2023*

KP1077 for Rare Sleep Disorders

- Phase 2 trial ongoing in idiopathic hypersomnia
- IND opened for narcolepsy

Financial

- Net revenue of \$2.9M for Q1 2023
- Cash, cash equivalents and investments of \$95.3M as of March 31, 2023
- Available capital expected extends cash runway into 2026

Leadership Appointments

- Sven Guenther, Ph.D. promoted to Chief Scientific Officer
- Christal Mickle promoted to Chief Development Officer
 - Effective June 1, 2023, will serve as interim CEO and President
- Josh Schafer joined as Chief Commercial Officer and EVP of Business Development

Arimoclomol – Innovative Product Addresses High Unmet Need in NPC



FIRST-IN-CLASS, ORAL TREATMENT

- Capsule can be swallowed whole, opened and mixed with foods/liquids or delivered through feeding tube
- Significant improvements in lysosomal and cellular function with arimoclomol treatment



EXTENSIVE CLINICAL EXPERIENCE WITH DEMONSTRATED SAFETY

- Studied in ten Phase 1, four Phase 2, and three Phase 2/3 trials
- No significant safety findings identified to date (500+ patients treated)
- Positive efficacy demonstrated in NPC trial (NPC-002)
- Data from the four-year open-label extension of Phase 2/3 trial showed trends consistent with the positive results from the 1 year double-blind phase



ADVANTAGEOUS REGULATORY DESIGNATION

- Orphan Drug Designation for NPC in U.S. and EU
- Fast-Track Designation, Breakthrough Therapy Designation, and Rare Pediatric Disease Designation from the FDA for NPC
- Eligible to receive Rare Pediatric Disease Priority Review Voucher if approved by FDA

Near-Term Opportunity to Commercialize and Retain Full Market Value

Launch arimoclomol with a small, focused commercialization effort which can be foundation for future rare disease product commercialization



- Small, nimble commercial team
- Lower marketing spend
- Patient advocacy relationships support adoption
- Commercial opportunities outside the U.S.
- Market entry through U.S. and E.U. EAPs

KP1077 – Novel Approach to Rare Sleep



SERDEXMETHYLPHENIDATE FOR RARE SLEEP DISORDERS

- Two dosing regimens being explored
 - Once daily at night
 - 2x daily-once in the morning and once at night
- Potential to address primary IH symptoms: sleep inertia and brain fog



IMPROVED SAFETY & TOLERABILITY OVER EXISTING TREATMENTS

- Greater tolerability and lower cardiovascular effects could allow for higher, more effective dosing (i.e. greater efficacy)
- No DDI potential with hormonal contraceptives; antidepressants



REGULATORY & IP ADVANTAGES

- Orphan Drug designation in IH
- Potentially eligible for other expedited approval pathways
- Solid IP through 2037 and potentially beyond
- SDX designated C-IV by DEA

Phase 1 clinical trial results confirmed cardiovascular safety risk of SDX improved vs. immediate-release and long-acting formulations of Ritalin® and SDX provided higher total exposure to d-MPH

KP1077 – Multiple Clinical Programs Targeting Rare Sleep Indications

KP1077 Represents a Potential “Portfolio in a Pill” Opportunity

IDIOPATHIC HYPERSOMNIA

- Lead KP1077 indication
- Ongoing Phase 2 clinical trial was initiated in December 2022
- Interim data from Phase 2 clinical trial expected as early as Q3 2023
- Top-line data expected as early as EOY 2023

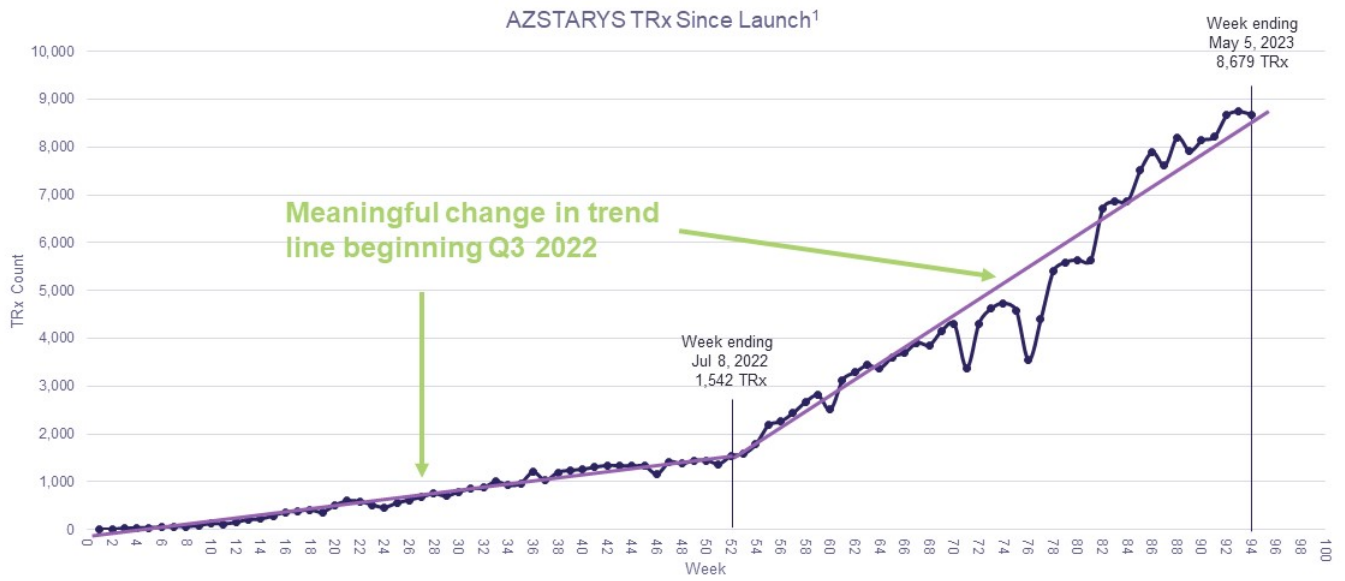
NARCOLEPSY

- IND opened for narcolepsy
- Second KP1077 indication allows Zevra to address two rare sleep indications that are underserved by currently available medications
- Will evaluate the potential to initiate a future Phase 3 trial in narcolepsy based on IH Phase 2 results
 - Data generated from IH program will expedite narcolepsy development timeline

IH Phase 2 results may support advancement into Phase 3 in narcolepsy

AZSTARYS® Prescription Trends Are Encouraging

Potential to achieve one or more net sales milestones based on current trend



¹ Source: Symphony Health, Metys™ Version 5.8.1, 2023



APPROVED BY THE U.S. FDA
IN MARCH 2021

INDICATED FOR

TREATMENT OF ADHD

IN PATIENTS 6 YEARS OF AGE AND OLDER



COMMERCIAL LICENSE TO
CORIUM, INC.

- Corium has achieved coverage with three largest PBMs
- Field sales force to 175 reps, supported by additional virtual sales reps to extend reach
- Increasing commercial team focus on adult market
- Significant market access success, with coverage of nearly 145 million lives, and preferred status for 35 million of those covered lives
- Growth trajectory of product continues
- Potential to reach one or more net sales-based milestones during 2023

Financial Position is a Source of Strength

Q1 2023 Financial Results:

- Net Revenue:
 - Q1 2023 was \$2.9M; derived primarily from French EAP reimbursements of \$2.3M and AZSTARYS royalties of \$0.6M
- Net Loss Attributable to Common Stockholders:
 - Q1 2023 was (\$11.8M), or (\$0.34) per basic and diluted share, driven primarily by R&D expense of \$8.8M, and G&A expense of \$6.8M, partially offset by net revenue of \$2.9M

Balance Sheet as of March 31, 2023:

- Cash, cash equivalents and investments was \$95.3M, a decrease of \$7.6M vs. Dec 31, 2022
- Repurchased 665,739 shares during Q1 2023 for \$3.4M at an average price of \$5.09 per share
- 33,881,804 shares of common stock outstanding, fully diluted shares outstanding of 49,307,811

Cash balance remains strong, with potential to realize milestone revenue

- Available cash, cash equivalents and investments expected to extend cash runway into 2026
 - Current operating plan includes the expected reimbursements from the French arimoclomol EAP, the full development of KP1077 through NDA submission and potential PDUFA, as well as investments needed to prepare for the potential U.S. launch of arimoclomol, if approved.
- Based on current prescription trend for AZSTARYS[®], we expect to achieve at least the first net sales milestone under the license agreement with Commave Therapeutics, SA
- Net revenue from French EAP program expected to continue at approximately \$2.0M per quarter throughout FY 2023 and beyond
- R&D investments for KP1077 will be higher during FY 2023 due to the ongoing Phase 2 trial, and the preparation for the potential initiation of a Phase 3 trial in IH

Significant Value Creation through Continued Execution

Advancing Pipeline

ARIMOCLOMOL

- Potential to re-file NDA as early as Q3 2023

KP1077

- Interim data from Phase 2 clinical trial expected as early as Q3 2023
- Top-line data expected as early as EOY 2023
- Potential to initiate Phase 3 trial in narcolepsy following IH Phase 2 trial results
- IND has been opened for narcolepsy

Realizing Commercial Opportunity & Retaining Value

- Launch arimoclomol with small, focused commercialization effort
- In-house commercial team provides foundation for future rare disease product commercialization
- Clinical, EAP and patient advocacy relationships support product adoption

Financial Strength & Growth

- Solid balance sheet supports development efforts and other pipeline expansion activities
- Available capital extends cash runway into 2026
- Net sales milestones and continued royalty revenue from AZSTARYS® add capital flexibility and support cash runway
- Anticipate ongoing quarterly revenue from arimoclomol French EAP reimbursements

Thank You.

