Q4 and FY 2022 Results

March 7, 2023

ZEVRA THERAPEUTICS

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Agenda

Strategic Overview, 2022 Highlights & Program Updates

Richard W. Pascoe, CEO

AZSTARYS® Commercial Update, 2022 Financial Results & 2023 Financial Guidance

R. LaDuane Clifton, CFO, Secretary & Treasurer

Upcoming Milestones & 2023 Outlook

Richard W. Pascoe, CEO

Q&A

Richard W. Pascoe, CEO
R. LaDuane Clifton, CFO, Secretary & Treasurer
Travis C. Mickle, President & Co-Founder
Christal Mickle, Chief Product Development Officer
Joshua Schafer, Chief Commercial Officer & EVP, BD

We are Zevra

ZEVRA THERAPEUTICS

Nimble and focused rare disease company with track record of success in overcoming drug development & regulatory challenges

Leveraging unique insights and regulatory strategies to forge pathways to success for transformational rare disease therapies

Well positioned for success with robust pipeline, world-class management and board and strong balance sheet

Our Strategic Evolution

ZEVRA THERAPEUTICS

EVALUATE » **STRATEGIZE** » **EVOLVE**

Responding to market dynamics, stakeholder demand, and changing technologies and circumstances to ensure **relevance, progress and prosperity**

Significant Value-Creation Opportunity in Rare Disease for Patients and Shareholders

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- Unique partnerships with patient communities
- Longer market exclusivity and less generic competition
- Lower cost of R&D
- Regulatory and financial incentives
- Shorter development timelines & smaller studies
- Small patient population served by specialist clinicians can be addressed with in-house commercial team

Why Zevra Therapeutics?

Zevra, Greek for zebra, is the internationally recognized symbol for the rare disease community

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Positioned for Success

Strategic focus on rare diseases	• Build a highly differentiated pipeline of development assets with multiple clinical and regulatory milestones • Focus on high-value areas with significant unmet needs in rare disease with potential to internally commercialize
Arimoclomol: treatment of Niemann-Pick disease Type C (NPC)	• NDA-stage drug candidate being developed for the treatment of NPC • “Capital efficient” financial structure; potential for positive cash flow; no shareholder dilution • NDA resubmission expected as early as Q3 2023; potential Zevra commercial candidate
KP1077: treatment of idiopathic hypersomnia (IH) and narcolepsy	• High-value opportunity with significant unmet need • Initiated IH Phase 2 Trial in December 2022 • Initiate Narcolepsy Phase 3 Trial post IH Phase 2 results
Other product opportunities	• Leverage prodrug platform to internally develop product candidates with significant potential value • Business development activities focused on complimentary clinical-stage rare disease assets
AZSTARYS® license	• Expanding launch of AZSTARYS provides ongoing revenue potential from royalties and milestones
Strong balance sheet	• Cash, cash equivalents and investments of **$102.9M** as of December 31, 2022 • Strong cash position supports development plan and other opportunities • Based on operating forecast, **cash runway expected to extend into 2026**

2022 Highlights

Arimoclomol for Niemann-Pick Type C

- **5/15:** Arimoclomol acquisition
- Completion of 4-year safety trial
- Ongoing collaborative dialogue and periodic meetings with the FDA

KP1077 for Rare Sleep Disorders

- **4/19:** Phase 1 SDX cardiovascular safety trial initiation
- **5/5:** IND submitted for Phase 2 study in idiopathic hypersomnia
- **9/28:** Phase 1 positive topline cardiovascular trial data
- **11/18:** Orphan drug designation in IH
- **12/21:** Phase 2 trial initiation in idiopathic hypersomnia

Financial

- **3/16:** One-time fee of $1.975M from Corium following FDA approval of ADLARITY®
- Net revenue of $10.5M for full year 2022
- Cash, cash equivalents and investments of $102.9M as of Dec. 31, 2022
- Available capital expected to extend cash runway into 2026

Leadership Appointments

- 11/28: Christopher Posner appointed independent director
- 8/9: Nichol Ochsner appointed VP, IR and Corp Comms

Q4 2022 and Recent Highlights

KP1077 for Rare Sleep Disorders
November 18, 2022
Orphan Drug Designation received for treatment of IH

Initiation of Phase 2 study in IH
KP1077 for Rare Sleep Disorders
December 21, 2022

Leadership Appointment
January 9, 2023
Matthew Plooster
Board Chairman
Richard W. Pascoe
Chief Executive Officer
Joshua Schafer,
Chief Commercial Officer and EVP of BD

Daniel Gallo, Ph.D.
SVP Medical Affairs & Advocacy
Abbi Maher, J.D.
VP of Legal Affairs
Leadership Appointment
January 31, 2023

Corporate
February 22, 2023
Company renamed Zevra Therapeutics, Inc.

Interim analysis of four-year safety study presented at WORLDSymposium suggest arimoclomol may reduce disease progression
Arimoclomol for Niemann-Pick Disease Type C
February 24, 2023

Corporate
February 28, 2023
Joined NORD Corporate Council

Began trading as ZVRA
Corporate
March 1, 2023

*Dates of corporate announcements

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Financial

Net revenue of **$2.3M** for Q4

Ended the year with **$102.9 million** of capital available on the balance sheet

World Class Leadership Team and Board

Decades of R&D and Commercialization Experience Driving Excellence in Rare Disease

Richard Pascoe
CEO

Travis Mickle, Ph.D.
President & Co-Founder

R. LaDuane Clifton
CFO, Secretary & Treasurer

Sven Guenther, Ph.D.
Chief Scientific Officer

Christal Mickle
Chief Product Development Officer & Co-Founder

Joshua Schafer
Chief Commercial Officer & EVP of BD

Board of Directors

Matthew R. Plooster
Chairman

Richard Pascoe

Travis Mickle, Ph.D.

Tamara A. (Seymour) Favorito

Joseph B. Saluri

David S. Tierney, M.D.

Christopher Posner

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ARIMOCLOMOL

For the Treatment of Niemann-Pick Disease Type C (NPC)

Arimoclomol – Innovative Product for a High Unmet Need

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FIRST-IN-CLASS, ORAL TREATMENT INTENDED FOR NPC

- Capsule formulation designed to be swallowed whole, opened to allow contents to be mixed with soft foods/liquids or delivered through a gastric feeding tube
- Nonclinical and clinical evidence demonstrated significantly improved lysosomal and cellular function with arimoclomol treatment

EXTENSIVELY RESEARCHED

- 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 results from NPC trial (NPC-002) and Phase 2 trial in Gaucher Disease (GD), both of which are lysosomal storage disorders

BENEFICIAL REGULATORY POSITIONING

- 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

Zevra expects to resubmit the NDA for arimoclomol in NPC as early as Q3 2023

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 products, including KP1077

1. Small & nimble commercial team
2. Lower marketing spend
3. Patient advocacy relationships support adoption
4. Market entry through U.S. and E.U. EAPs
5. Commercial opportunities outside the U.S.

SDX PRODUCT CANDIDATE: KP1077

For the Treatment of Idiopathic Hypersomnia (IH)

KP1077 – Multiple Clinical Programs Targeting Rare Sleep Indications

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KP1077 Represents a Potential “Portfolio in a Pill” Opportunity

IDIOPATHIC HYPERSOMNIA

- Lead KP1077 indication
- Investigational New Drug (IND) application cleared by FDA
- 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 by EOY 2023

NARCOLEPSY

- Second KP1077 indication would allow Zevra to address two rare sleep indications that are underserved by currently available medications
- Evaluate the potential to initiate narcolepsy Phase 3 trial based on IH phase 2 results
  - Seek to leverage key data points from IH program to expedite narcolepsy development

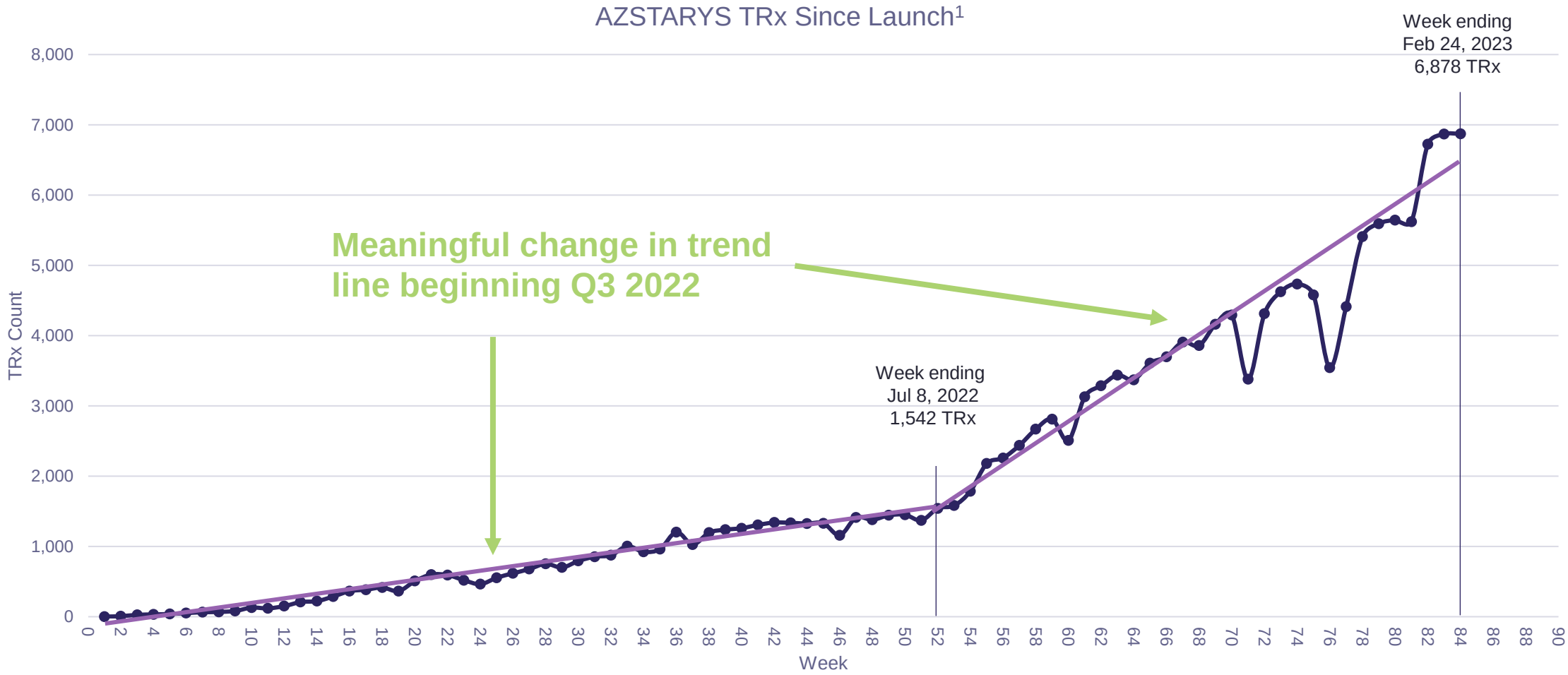
IH Phase 2 results may support advancement into Phase 3 in narcolepsy

AZSTARYS® Commercialization Update

d-Methylphenidate Prodrug Product for the Treatment of ADHD

AZSTARYS® Prescription Trends Are Encouraging

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



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

AZSTARYS® Provides a Foundation

Royalties from legacy develop/out-license model may not be a strong growth driver in short or medium term

	Quarterly TRx[1]	Cumulative TRx	Actual Royalties
Q3 2021	497	497	$0.03M
Q4 2021	5,189	5,686	$0.06M
Q1 2022	8,710	14,396	$0.08M
Q2 2022	15,130	29,526	$0.13M
Q3 2022	25,418	54,944	$0.24M
Q4 2022	53,811	108,755	$0.40M
Q1 2023 (thru Feb 24)	55,272	164,027	TBD

- Trend line improvement noted in Q3 2022, with coverage at three largest PBMs and ~175 field reps in the field (as reported by Corium, Inc.)
- Potential for FY 2023 to benefit from added focus on adult ADHD market
- Royalties are meaningful, but modest
- Sales milestones, if achieved, improve return

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

FINANCIAL UPDATE

Financial Position is a Source of Strength

Q4 and FY 2022 Results:

- Net Revenue:
  - Q4 2022 was $2.3M; FY 2022 was $10.5M, derived primarily from the French EAP program, royalties and consulting service fees
- Net Loss Attributable to Common Stockholders:
  - Q4 2022 was ($9.0M), or ($0.26) per basic and diluted share, driven primarily by R&D expense of $6.4M, and G&A expense of $5.1M, partially offset by net revenue of $2.3M
  - FY 2022 was ($41.5M), or ($1.20) per basic and diluted share, driven primarily by R&D expense of $19.6M, G&A expense of $15.3M, and a one-time non-cash charge of $17.7M for in-process R&D from the arimoclomol acquisition, partially offset by net revenue of $10.5M
    - Non-GAAP FY 2022 net loss excluding the one-time non-cash charge of $17.7M was ($23.9M), or ($0.69) per basic and diluted share

Balance Sheet as of Dec 31, 2022:

- Cash, cash equivalents and investments was $102.9M, a decrease of $4.5M vs. Sep 30, 2022
- Common shares outstanding of 34,540,304, fully diluted shares outstanding of 47,088,184

2023 Financial Guidance

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.

Outlook for 2023 and Beyond

Multiple Growth Catalysts in 2023

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Arimoclomol

- Potential to re-file NDA as early as Q3 2023
- Anticipate ongoing quarterly revenue from French EAP reimbursements

KP1077 Development Program

- Interim data from Phase 2 clinical trial expected as early as Q3 2023
- Top-line data expected by EOY 2023
- Potential to initiate phase 3 trial in narcolepsy following IH phase 2 trial results
- Expect to file IND in Q2 2023 for narcolepsy

Strong Balance Sheet to Support Value Creation

- Solid balance sheet supports development efforts and other pipeline expansion activities
- Available capital extends cash runway into 2026

AZSTARYS®

- Potential to realize sales milestones and continued royalty revenue from AZSTARYS®
- Adds capital flexibility and potential to further extend cash runway

Q&A

Thank You.

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