Q1 2023 Results

May 15, 2023

ZEVRA THERAPEUTICS

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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

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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

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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

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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

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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

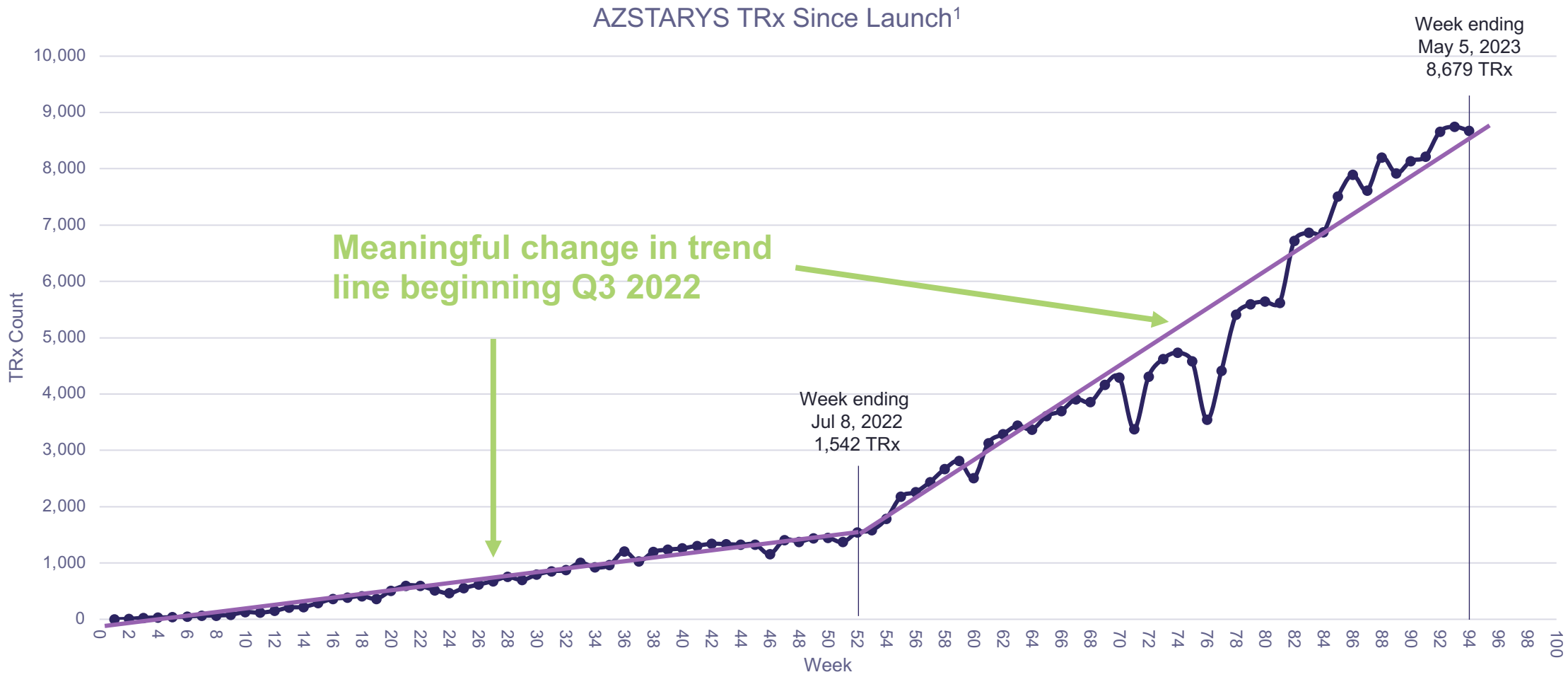
NARCOLEPSY

- 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

AZSTARYS®

Commercial Product Delivering Value

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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

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 in IH

Significant Value Creation through Continued Execution

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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.

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