Corporate Presentation

March 2023

ZEVRA THERAPEUTICS

Cautionary Note Regarding Presentation Information

This presentation 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, including without limitation and 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 statements regarding the promise and potential impact of our preclinical or clinical trial data, including without limitation the timing and results of any clinical trials or readouts, the timing or results of any Investigational New Drug applications and NDA submissions, including the resubmission of the NDA for arimoclomol, communications with the FDA, the potential uses or benefits of arimoclomol, KP1077, SDX or any other product candidates for any specific disease indication or at any dosage, the potential benefits of any of’ Zevra’s product candidates, the success or timing of the launch or commercialization of AZSTARYS® or any other products or related sales milestones, the sufficiency of cash to fund operations, our plans or ability to seek funding, our plans with respect to our share repurchase program, and our strategic and product development objectives. These forward-looking statements are based on information currently available to Zevra and its current plans or expectations and are subject to a number of known and unknown uncertainties, risks and other important factors that may cause our actual 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 Annual Report on Form 10-K for the year ended December 31, 2021, as updated by the Quarterly Report on Form 10-Q for the three months and nine months ended September 30, 2022, and Zevra’s 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 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.

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

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, CPA
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

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**

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

ZEVRA THERAPEUTICS

Financial

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

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

ZEVRA THERAPEUTICS

ARIMOCLOMOL

For the Treatment of Niemann-Pick disease type C (NPC)

Arimoclomol - Expanding Pipeline Targeting Rare Diseases

Aligns with strategy to build value through the development and commercialization of novel treatments for rare diseases

Niemann-Pick disease type C

- Ultra-rare progressive, disabling and fatal lysosomal storage disorder
- No approved treatments exist in the U.S. for NPC

High Upside Opportunity

- NDA-stage investigational drug candidate
- Zevra has expertise in NDA resubmissions following CRLs

Favorable Acquisition Terms

- “Capital efficient” financial structure with potential for positive cash flow and no shareholder dilution

Early Access Programs

- Available to NPC patients in the U.S., France, Germany and other European countries
- French EAP expected to generate annual net revenue of ~$8M (gross revenue of ~$12M)

About Niemann-Pick Disease Type C (NPC)[1]

ZEVRA THERAPEUTICS

PROGRESSIVE LYSOSOMAL STORAGE DISORDER

- Characterized by an inability of the body to transport cellular cholesterol and lipids
- Leads to dysfunction in organs such as the brain, spleen and liver
- Disease progression is irreversible in all patients and ultimately fatal
- Loss of neuro-cognitive function adversely impacts the daily lives of patients

SIGNIFICANT UNMET NEED

- NPC can range from a fatal disorder within the first few months after birth (neonatal period), to a late onset, chronic progressive disorder that remains undiagnosed well into adulthood
- Mean age of death in NPC patients is 13 years[2]
- No approved treatments exist in the U.S. for NPC

ULTRA RARE

- NPC is estimated to occur in 1 in 100,000-120,000 live births
- Estimated 1,800 patients diagnosed in the U.S. and E.U

Source: (1) https://rarediseases.org/
(2) Bianconi, 2019

Arimoclomol – Innovative Product for a High Unmet Need

ZEVRA THERAPEUTICS

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

Overview of Regulatory Pathway in the U.S. – NDA Resubmission Process

COMPLETE RESPONSE LETTER

- Orphazyme received a Complete Response Letter (CRL) from the FDA on Jun 17, 2021, regarding NDA for arimoclomol for the treatment of NPC
  - The FDA identified three issues:
    1) Additional evidence needed to support use of the NPCCSS as the primary instrument in measuring NPC disease progression
    2) Required additional analysis related to how missing data is handled for statistical analysis
    3) Required additional support and data related to confirmatory evidence of efficacy
- **The FDA did not request additional efficacy data in the CRL**

TYPE A END-OF-REVIEW MEETING

- Type A End-of-Review Meeting was held on Oct 13, 2021:
  - *FDA agreed* to allow a reanalysis of the 5-domain NPCCSS removing the cognition domain
  - *FDA agreed* to a rescoring and a reassessment of the swallowing domain including a qualitative study to further validate that domain
  - *FDA agreed* to further discussions regarding the primary instrument, NPCCSS, the analysis of the data after rescoring and the need for additional confirmatory evidence prior to resubmission of the NDA

Path to Resubmission and Approval Progressing

Zevra has significant experience with challenging regulatory situations, including two FDA product approvals that followed initial CRLs

RECENT ACTIVITIES

- Continuing to have an ongoing collaborative dialogue and periodic meetings with the FDA
  - Intended to ensure an optimal NDA data package that addresses all issues in the CRL
- Advancing activities to bolster arimoclomol NDA with confirmatory evidence for resubmission to the FDA
  - Working to analyze and process the new data generated since the CRL
  - This includes data obtained from a 4-year arimoclomol safety study and safety data from other clinical trials with arimoclomol

REGULATORY OUTLOOK

- Throughout this process, no new issues or concerns have been raised by the FDA
  - No new efficacy trial has been proposed by FDA
- We believe there is a viable pathway to enable a successful NDA resubmission and subsequent approval for arimoclomol in NPC
  - Path may include, if necessary, additional non-clinical or clinical studies, a Federal Dispute Resolution Request (FDRR) and/or an advisory committee (ad com) requested by either FDA or Zevra

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

Overview of Regulatory Pathway in the U.S. – NDA Resubmission Process

RARE PEDIATRIC DISEASE PRIORITY REVIEW VOUCHER

- Upon approval, Zevra would be eligible to receive a Rare Pediatric Disease Priority Review Voucher
  - Estimated value of ~$100M based on recent PRV sales
  - Program could eventually end, making these vouchers more scarce

EARLY ACCESS PROGRAMS

- Arimoclomol is generating revenue from French EAP reimbursements
  - French program is the only system that reimburses for treatment prior to formal approval; rate is set by the Sponsor
- **Global EAP programs represent the potential first adopters of arimoclomol post-approval**
  - Early access and other compassionate use programs are active in the U.S., Germany, France, Italy, Denmark, Switzerland and the U.K.

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.

ZEVRA THERAPEUTICS

SDX PRODUCT CANDIDATE: KP1077

For the Treatment of Idiopathic Hypersomnia (IH)

KP1077 – Product Candidate Overview

Zevra is advancing KP1077 as a potential therapeutic treatment for Idiopathic Hypersomnia (IH)

Serdexmethylphenidate

- 100% SDX with multiple dosing options
- SDX granted Orphan Drug Designation for IH by FDA
- SDX designated C-IV by DEA

Dosing Addresses Symptoms

- Dosed either 1x daily at bedtime or 2x daily at bedtime and at waking
- Potential to address primary IH symptoms: sleep inertia and brain fog

Regulatory & IP Advantages

- Orphan Drug designation in IH
- Eligible for Fast-Track and Breakthrough Therapy designation
- Solid IP through 2037 and potentially beyond

Improved Safety & Tolerability

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

Idiopathic Hypersomnia

MARKET SIZE

- There are 10.3 IH patients per 100,000 people in the US[1]
- ~37,000 diagnosed IH patients actively seeking treatment[2]
- Total population may be much larger (not seeking treatment, undiagnosed, misdiagnosed)

SYMPTOMS

- Symptoms are highly debilitating and can be **more debilitating than narcolepsy**
  - Chronic daytime sleepiness
  - Long and unrefreshing naps
  - Extreme difficulty waking
  - Severe brain fog
  - Excessively long sleep times (~25% of patients “long sleepers”, >10hrs)

RARE

- IH patients report memory problems, errors in habitual activities, mind blank and attention problems as part of their disability
  - KOLs identified depression as a common comorbidity encountered with patients
  - Patients rated current medication effectiveness as poor (5.4 on a 10-point scale)[3]

Sources: (1) https://doi.org/10.1093/sleep/zsy061.624
(2) https://www.sleepcountshcp.com/what-is-idiopathic-hypersomnia
(3) https://www.sleepcountshcp.com/idiopathic-hypersomnia-treatment-options

Phase 2 Clinical Trial Investigating KP1077 for the Treatment of IH

Multi-center, dose-optimizing, double-blind, placebo-controlled, randomized-withdrawal study to evaluate safety and efficacy of KP1077, as well as to assess the symptoms and severity of “brain fog”

PHASE 2 TRIAL (N=48)

Part 1:

- Five-week open-label titration phase
- Patients optimized to one of the four doses of SDX (80, 160, 240, or 320 mg/day)

Part 2:

- Two-week randomized, double-blind, withdrawal phase
- 2/3 receive active; 1/3 placebo
- 50% receive single daily dose; 50% receive half daily dose upon awakening and at bedtime

PRIMARY ENDPOINT

- Safety and tolerability of SDX

MAJOR SECONDARY ENDPOINT

- Change in Epworth Sleepiness Scale (ESS) total score

ADDITIONAL EXPLORATORY ENDPOINTS

- Patient Global Impression of Severity (PGI-S)
- Clinical Global Impression of Severity (CGI-S)
- Change in total score on the Idiopathic Hypersomnia Severity Scale (IHSS)
- New scale to assess the symptoms and severity of “Brain Fog”

KP1077: Addressing Cardiovascular Stimulant Comorbidities

Many comorbidities and patient demographics complicate treatment, including cardiovascular issues

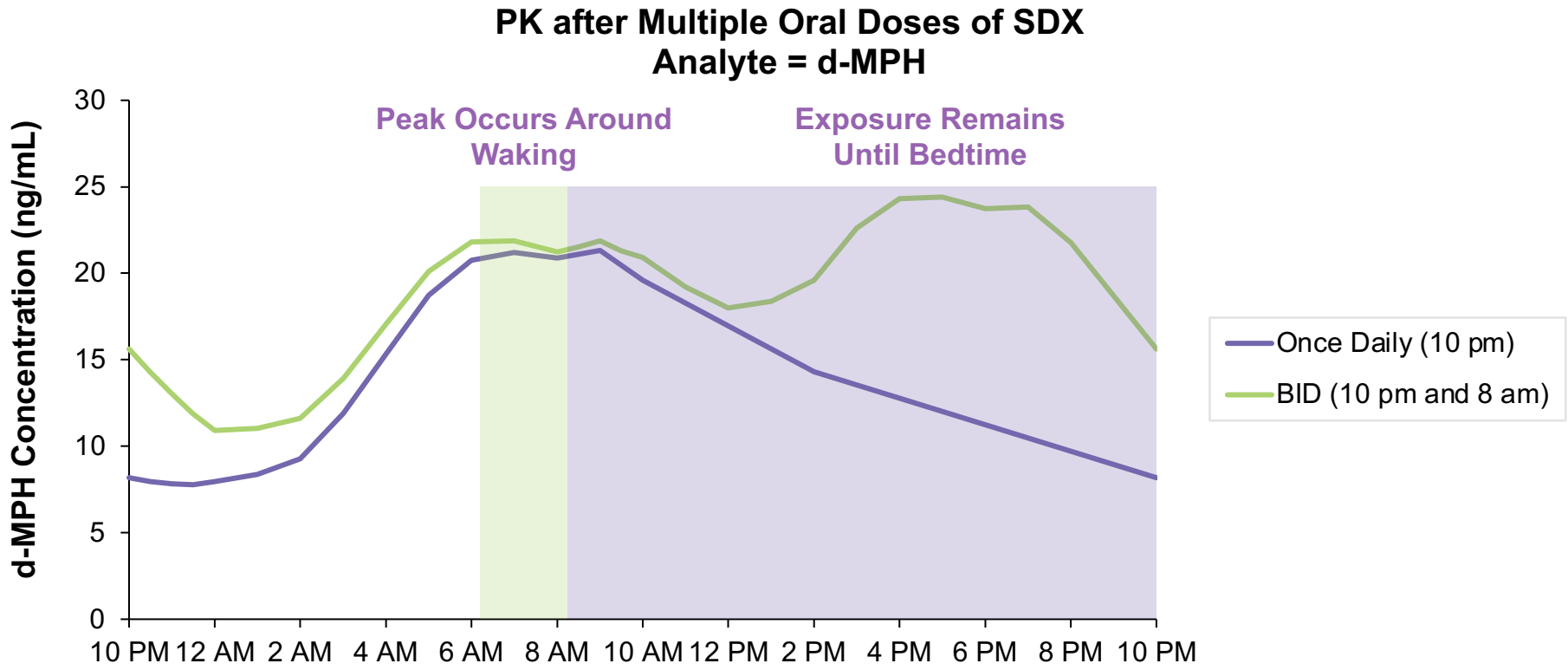
- *Brain fog in IH is so debilitating that current, tolerable stimulant treatment doses are inadequate*:
  - The ability to dose higher with fewer negative side-effects, including those associated with blood pressure (BP) and heart rate (HR), compared to current off-label treatments have the potential to more adequately address brain fog
- *High BP and HR increases are associated with other stimulant treatments; could lead to dose limitations, discontinuation or contraindication (est. ~50% of US population has HBP)*[1]
  - Due to the unique pharmacokinetic profile of SDX, KP1077 may be demonstrably better than current stimulants including MPH products with regards to BP and HR

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

- Phase 2 trial doses should be well tolerated and potentially address the most problematic symptoms of IH

(1) https://www.cdc.gov/bloodpressure

Predicted Pharmacokinetics for Two Potential Dosing Regimens of SDX (Once Daily or B.I.D) Represent Significant Potential Improvements in IH Symptom Treatment



Plasma concentrations were estimated based on data collected in study KP879.101

Predicted PK is shown for steady state of 240 mg SDX based on single oral dose of 240 mg SDX CI in KP879.101

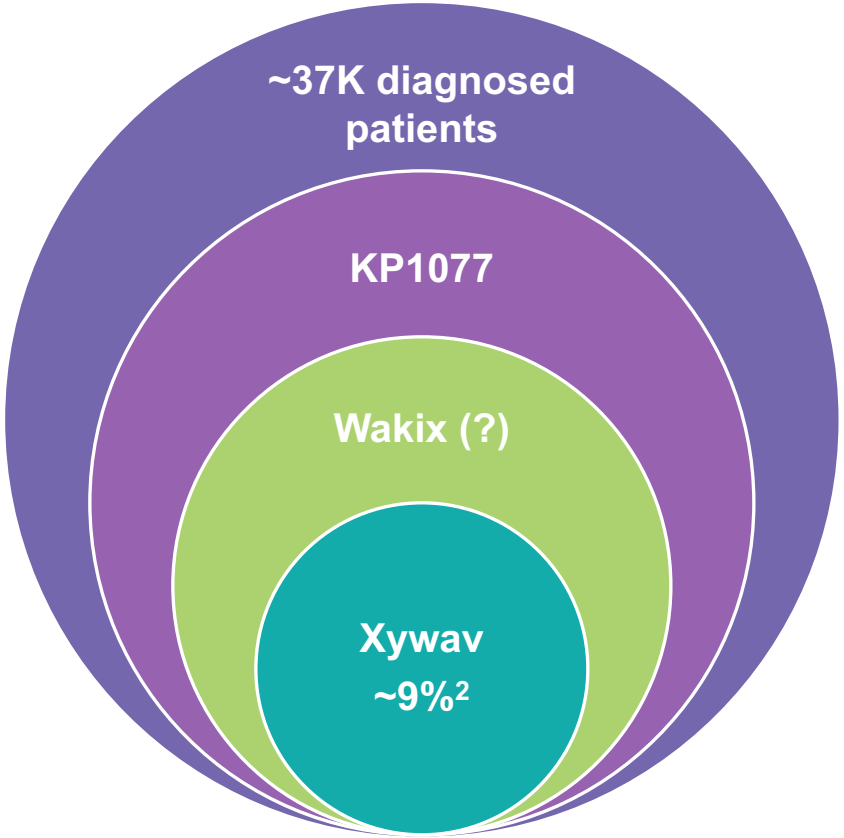
KP1077 Could Capture a Large Share of the IH Market Share

Competitive Products in IH

- Xywav® received FDA approval in August 2021 as the first therapy for IH
  - According to analysts, Xywav projected sales are ~$300 million for IH by the end of 2025[1]
- Wakix® currently enrolling patients in a Phase 3 clinical trial in IH

KP 1077 Potential Differentiation

- **MOA and improved efficacy of KP1077:** positioned as a monotherapy and combination use with oxybate (Xyrem, Xywav or others)
- **KP1077 safety profile**: Schedule IV, lack of drug-drug-interaction with hormonal contraceptives which is an issue with modafinil, reduced risk of adverse events compared to current off-label IH therapies
- **Xywav barriers to uptake**: clinical trial discontinuation rate of ~11% due to treatment emergent adverse events, boxed warning for CNS depression, abuse and misuse potential, REMS program, negative stigma associated with GHB[2]
- **Wakix barriers to uptake:** DDI, especially with antidepressants and antihistamines



Sources: (1) https://investor.jazzpharma.com/investors/events-presentations
(2) https://www.reuters.com/business/healthcare-pharmaceuticals/us-fda-approves-jazz-pharmas-drug-excessive-daytime-sleepiness-2021-08-12/

KP1077 – Multiple Clinical Programs Targeting Rare Sleep Indications

ZEVRA THERAPEUTICS

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

ZEVRA THERAPEUTICS

AZSTARYS®

d-Methylphenidate Prodrug Product for the Treatment of ADHD

Corium, Inc. – AZSTARYS® Commercialization

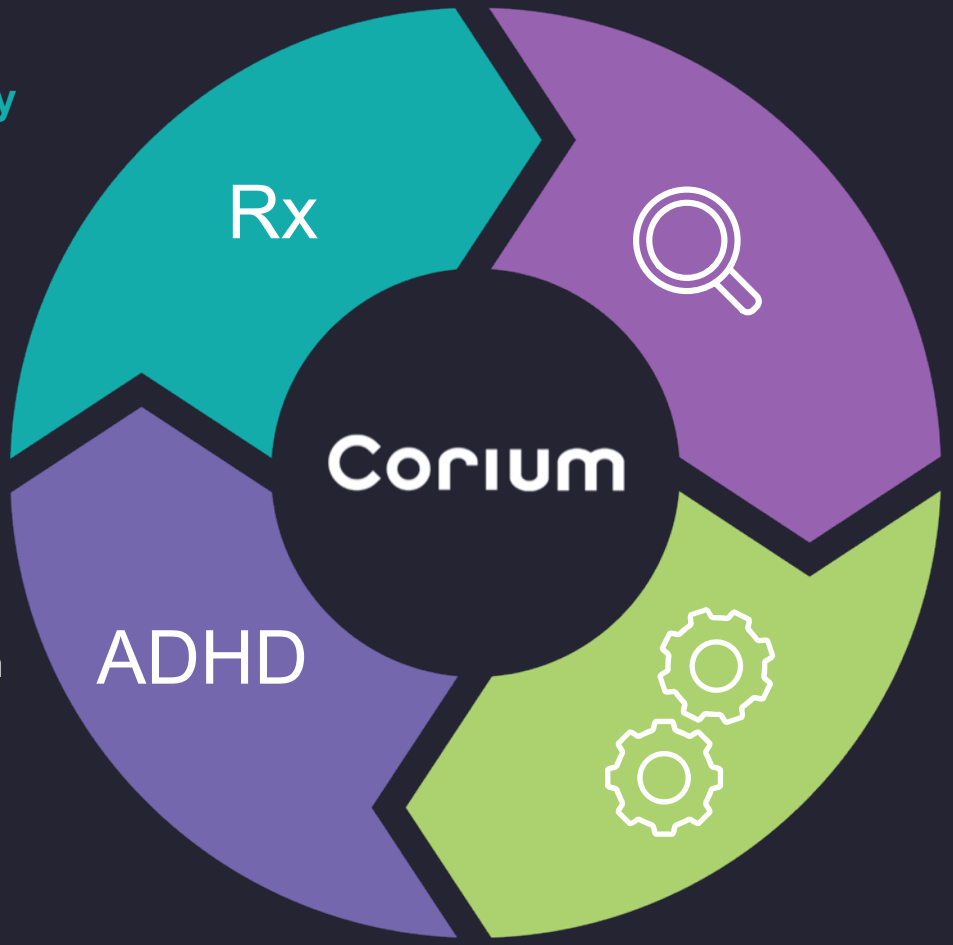
ZEVRA THERAPEUTICS

End-to-End Pharma Company

- Developing and commercializing innovative CNS therapies
- Owned by affiliate of Gurnet Point Capital
- Offices and manufacturing facility in Grand Rapids, MI and Boston, MA

ADHD Expertise

- Led by Perry Sternberg and team with broad ADHD expertise, including former Shire executives responsible for helping Vyvanse® achieve blockbuster status



Commercial Focus

- Two CNS product approvals in past 12 months
- AZSTARYS (March 2021) and ADLARITY® (March 2022 for Alzheimer's dementia)

CDMO Capabilities

- Transdermal development and manufacturing expertise
- Developed and manufacturing several consumer and FDA approved drug products

AZSTARYS® Product Highlights

FIRST & ONLY APPROVED

METHYLPHENIDATE-BASED DRUG CONTAINING SDX

APPROVED BY THE U.S. FDA

IN MARCH 2021

70%

Prodrug of d-MPH
(serdexmethylphenidate, or SDX)

CO-FORMULATED WITH **30%** IMMEDIATE RELEASE D-MPH

- **AZSTARYS® features and benefits**
  - Indicated for the treatment of ADHD in patients 6 years of age and older
  - Can be administered with or without food
  - Capsule can be opened and sprinkled in applesauce or water
  - In a 12-month study, no clinically significant changes in height or weight compared to normal growth
  - **Product is a Schedule II drug, with SDX component being Schedule IV**
  - LS mean change in SKAMP-C Score from baseline was different at all timepoints from 30 minutes to 13 hours post-dose for AZSTARYS vs. placebo
- No generic equivalent product
- Composition-based patent expires in 2037; NCE status granted; PTE and pediatric exclusivity possible as well

AZSTARYS® - U.S. Commercial Launch Update

RX GROWTH

- Steady growth in prescriptions during market introduction phase in both breadth and depth of prescribing
- Increasing number of pharmacies ordering AZSTARYS based on geographic areas in which Corium places sales representatives

NATIONAL LAUNCH PROGRESS

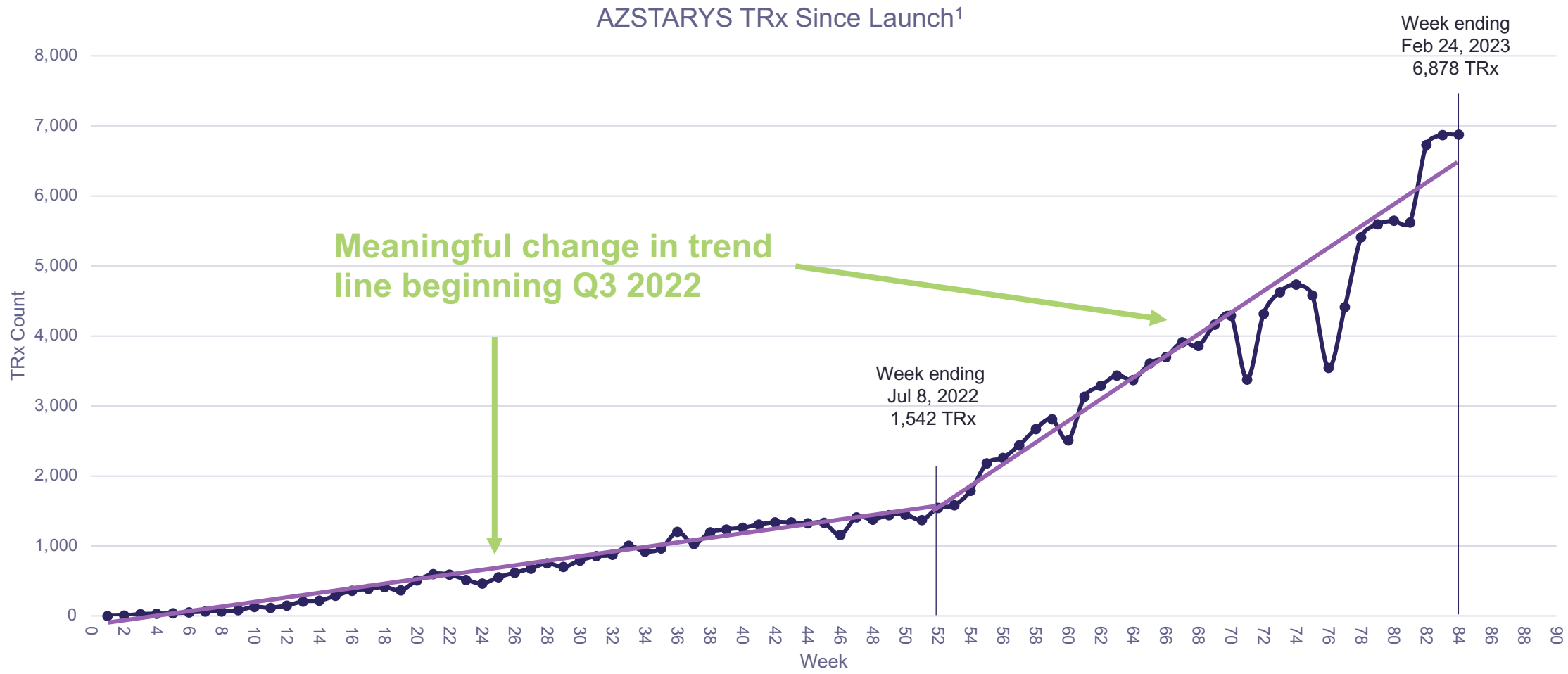
- Initial regional launch in 2021 and early 2022 focused on geographies with product coverage
- As of July 2022, National field team deployed comprised of ~175 field sales reps
- Held first AZSTARYS National Sales Meeting in July 2022 in connection with National launch
- Significant market access success, with coverage of almost 145 million lives and preferred status for 35 million of those covered lives

ADULT ADHD MARKET

- Increasing commercial team focus on adult market
- With Takeda pulling back on Vyvanse field sales promotion we are expanding call deck from just pediatric targets into adults

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

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

Outlook for 2023 and Beyond

Multiple Growth Catalysts in 2023

ZEVRA THERAPEUTICS

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

Thank You.

ZEVRA THERAPEUTICS