Corporate Presentation

May 2023

ZEVRA THERAPEUTICS

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Transformational Therapies for Rare Diseases

Positioned for Success	• Robust pipeline with multiple near-term clinical and regulatory milestones • Nimble and focused team with track record of success in overcoming drug development & regulatory challenges including two FDA approvals following CRLs • Strong balance sheet
Arimoclomol for Niemann-Pick disease type C (NPC)	• Orally-delivered, first-in-class investigational product candidate • NDA resubmission expected as early as Q3 2023 • Plans to commercialize with small in-house team
KP1077 for idiopathic hypersomnia (IH) and narcolepsy	• Lead prodrug candidate for idiopathic hypersomnia (IH) and narcolepsy type I & II • Initiated IH Phase 2 Trial in December 2022 • Plans for Phase 3 Trial post IH Phase 2 results
Creating and retaining commercial value	• Building commercial enterprise for focused arimoclomol commercialization effort • In-house commercial team provides foundation for future rare disease product commercialization • EAPs and patient advocacy relationships support product adoption at launch • Lifecycle management through prodrug technology

Strong Financial Position

Ended Q1 2023 with **$95.3 million** of capital available on the balance sheet

- **Cash runway expected to extend into 2026**
- Net revenue of **$2.9M** for Q1 2023
- **AZSTARYS® royalties & milestones bolster capital position**

Pipeline and Marketed Products

PRODUCT CANDIDATE	INDICATION	PHASE 1	PHASE 2	PHASE 3	NDA SUBMISSION	MILESTONES
NIEMANN-PICK DISEASE TYPE C (NPC) PROGRAM						
Arimoclomol *Orally-delivered, first-in-class investigational product candidate*	**Niemann-Pick Disease Type C (NPC)**					Arimoclomol NDA resubmission to FDA as early as Q3 2023; potential Zevra commercial candidate
RARE SLEEP DISORDERS PROGRAM						
KP1077 *Lead prodrug candidate for sleep disorders*	**Idiopathic Hypersomnia (IH)**					Interim Phase 2 IH efficacy and safety data expected as early as Q3 2023
	Narcolepsy					Initiate the first of several Phase 1 clinical trials to support both indications

MARKETED PRODUCT	INDICATION	COMMERCIAL PARTNER	MILESTONES
ADHD COMMERCIAL PRODUCT			
AZSTARYS® *serdexmethylphenidate and dexmethylphenidate*	**Attention Deficit Hyperactivity Disorder (ADHD) in patients age six and older**	Corium Inc.	Potential to reach one or more milestones during 2023 and continued royalty revenue

Active Member of Rare Disease Community

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Relationships with advocacy organizations support program advancement

NORD®

nnpdf
NATIONAL NIEMANN PICK DISEASE FOUNDATION

PROGRESS TOGETHER
Inpda
International Niemann-Pick Disease Alliance

hypersomnia foundation.org®

HOPE FOR MARIAN

Sleep consortium

ARA PARSEGHIAN MEDICAL RESEARCH FUND
UNIVERSITY OF NOTRE DAME

np uk

Firefly FUND
SHINING A LIGHT ON NPC1

inpdr

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ARIMOCLOMOL

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

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

ULTRA RARE

- Estimated to occur in 1 in 100,000-120,000 live births[1]
- Approximately 1,800 patients diagnosed in the U.S. and E.U

PROGRESSIVE LYSOSOMAL STORAGE DISORDER

- Characterized by an inability of the body to transport cellular cholesterol and lipids
- Leads to organ dysfunction in brain, spleen and liver
- Ranges from fatal within the first few months after birth (neonatal period), to late onset, chronic progressive disorder that remains undiagnosed well into adulthood

SIGNIFICANT UNMET NEED

- Neuro-cognitive decline adversely impacts daily living
- Irreversible and fatal disease
- Mean age of death in NPC patients is 13 years[2]
- No approved treatments exist in the U.S. for NPC

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

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

Creating Value for Patients and Shareholders

EARLY ACCESS PROGRAMS PROVIDE PATIENT DATA, REVENUE STREAM AND FOUNDATION FOR COMMERCIAL LAUNCH

- **Data from patients in EAP support re-submission of NDA**
- **Revenue from French EAP reimbursements**
  - The only system that reimburses for treatment prior to formal approval; rate is set by the Sponsor
- **Potential first adopters of arimoclomol post-approval**
  - Early access and other compassionate use programs active in the U.S., Germany, France, Italy, Denmark, Switzerland and the U.K.

RARE PEDIATRIC DISEASE PRIORITY REVIEW VOUCHER

- **Upon approval, Zevra eligible to receive a Rare Pediatric Disease Priority Review Voucher**
  - Estimated value of ~$100M
  - Program could eventually end, making these vouchers more scarce

Advancing Arimoclomol Toward NDA Resubmission as Early as Q3 2023

June 17, 2021

FDA Complete Response Letter recommended to provide the following information:

- Additional evidence to support use of NPCCSS as primary instrument in measuring NPC disease progression
- Additional analyses/data to support findings from the Phase 2/3 trial (including the handling of missing data for statistical analysis)
- Additional support and data related to confirmatory evidence of efficacy

NO REQUEST FOR ADDITIONAL EFFICACY DATA

October 13, 2021

Outcome of Type A End-of-Review Meeting with FDA:

- Analysis of the 4-domain NPCCSS (without the cognition domain)
- Conduct of a qualitative study to further validate the swallowing domain, including the potential of rescoring
- Further discussions regarding the primary instrument, NPCCSS, and need for additional confirmatory evidence prior to NDA resubmission

May 15, 2022

Acquisition of Arimoclomol from Orphazyme

February 24, 2023

Data from the four-year open-label extension of the Phase 2/3 clinical trial shows trends consistent with the 1-year double-blind phase of the trial

- Ongoing dialogue and meetings with FDA to ensure optimal NDA data package that addresses all issues in CRL
- No new issues or concerns
- To date, no new efficacy trial has been proposed by FDA

Q3 2023

- Path to NDA resubmission and subsequent approval for arimoclomol in NPC may include:
  - Additional non-clinical or clinical studies
  - Federal Dispute Resolution Request (FDRR)
  - Advisory committee (ad com) requested by FDA or Zevra

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**

SDX PRODUCT CANDIDATE: KP1077

For the Treatment of Rare Sleep Disorders

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

Need for Better Treatments for Idiopathic Hypersomnia

RARE

- 10.3 IH patients per 100,000 people in the US[1]
- ~37,000 diagnosed & actively seeking treatment[2]
- Total population may be much larger

SYMPTOMS HIGHLY DEBILITATING

- Chronic daytime sleepiness
- Long and unrefreshing naps
- Extreme difficulty waking
- Excessively long sleep times (~25% of patients “long sleepers”, >10hrs)
- Brain fog, memory problems, errors in habitual activities, mind blank and attention problems

CURRENT TREATMENTS DON’T ADDRESS NEED

- Patients rated current medication effectiveness as poor (5.4 on a 10-point scale)[3]
- Tolerable stimulant treatment doses are inadequate to treat brain fog
- Comorbidities complicate treatment (cardiovascular and patient demographics)
- Potential DDI with contraceptives, antidepressants, antihistamines

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

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

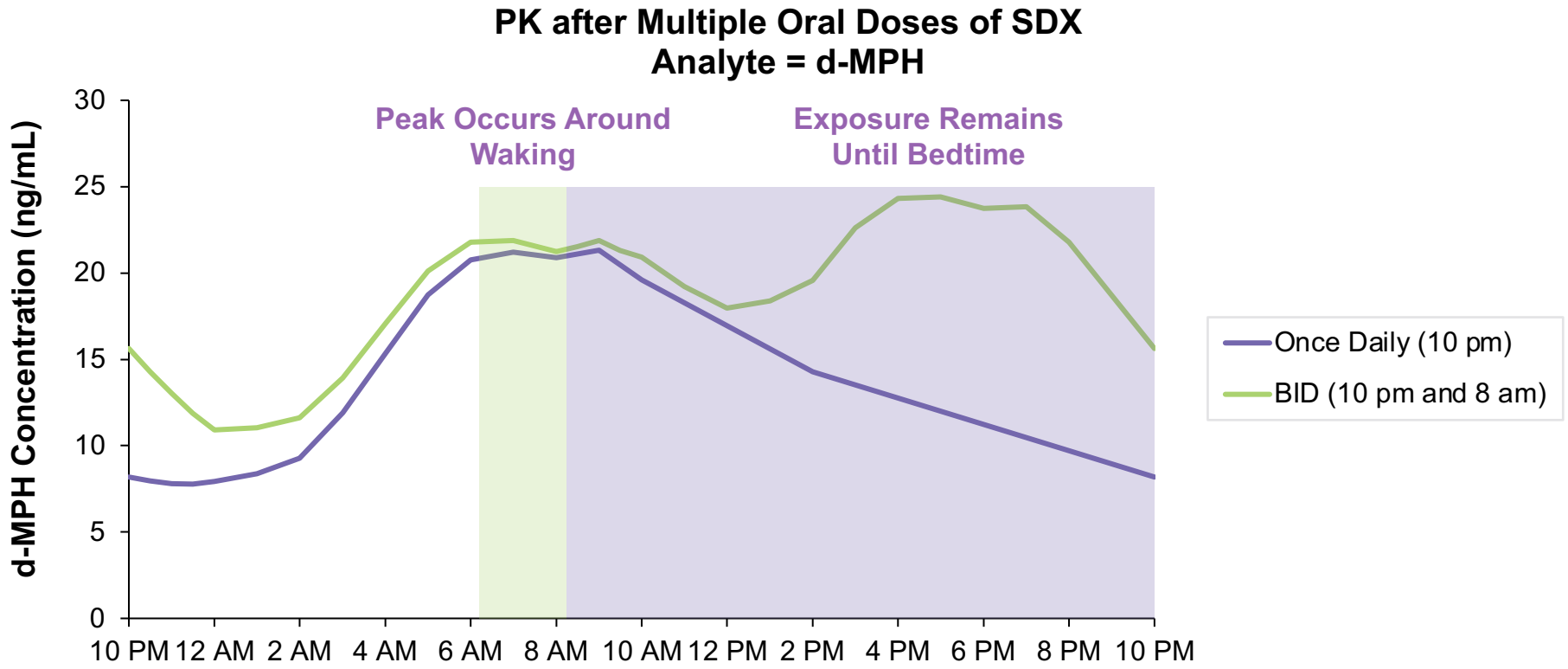
- Greater tolerability and lower cardiovascular effects could allow for higher, more effective dosing (i.e. greater efficacy)
- No DDI potential with hormonal contraceptives; antidepressants

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

Two Dosing Regimens Being Explored to Achieve Sustained Symptom Management



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

Phase 2 Clinical Trial of KP1077 in 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 Could Capture Significant IH Market Share

IH is characterized by excessive sleepiness, significant sleep inertia and brain fog

Current products primarily treat sleepiness and have limitations in treating all symptoms of IH

- Xywav® received FDA approval in August 2021 as first therapy for IH
- Wakix® currently enrolling patients in a Phase 3 clinical trial in IH

KP1077 differentiated profile has potential to better meet unmet needs of

- Improved safety profile and reduced risk of adverse events compared to current IH therapies
- Reduced risk vs other IH drugs of negative interaction with antidepressants and antihistamines
- Potential use as monotherapy or in combination use with oxybate (Xyrem®, Xywav or others)

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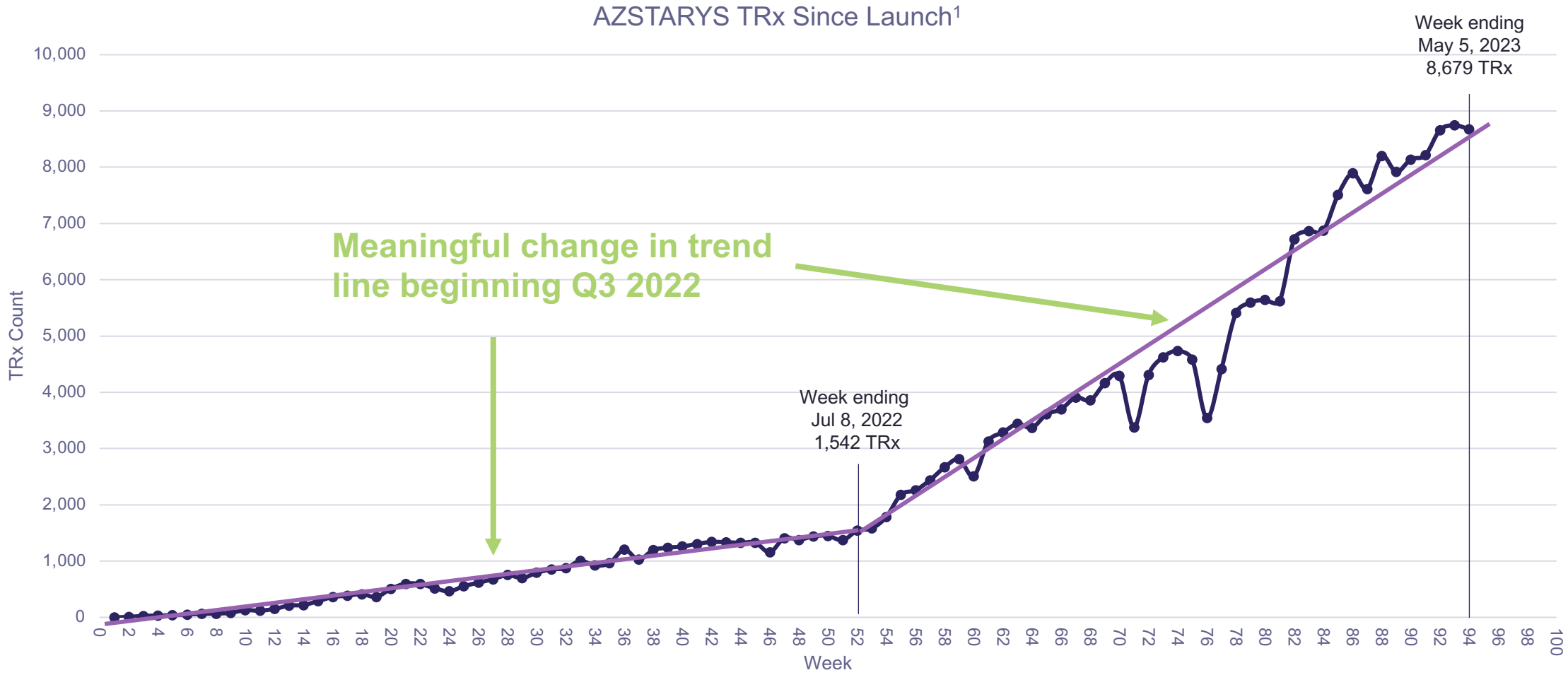
AZSTARYS®

d-Methylphenidate Prodrug Product for the Treatment of ADHD

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

ZEVRA THERAPEUTICS

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

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

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