



**KemPharm**<sup>INC</sup>

**Management Presentation**

**January 2023**

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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 KemPharm’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 KemPharm 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 KemPharm’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 KemPharm’s other filings with the Securities and Exchange Commission.

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# KEMPHARM VALUE PROPOSITION

Innovative rare disease company with a proven regulatory track record

Revenue-generating assets with significant commercial potential in areas of high unmet need

Strong balance sheet which is expected to fund operations and U.S. commercial build into 2026

# KemPharm: Recent Highlights

- ✓ Completion of the 4-year safety trial
- ✓ Ongoing collaborative dialogue and periodic meetings with the FDA
- ✓ Working to amass and characterize the new data generated since the CRL
- ✓ NDA refiling targeted as early as Q3 2023

## Arimoclomol

## KP1077 Development Program

- ✓ Phase 2 trial in IH initiated; Interim efficacy and safety data expected as early as Q3 2023
- ✓ SDX granted Orphan Drug Designation by FDA for treatment of IH
- ✓ Phase 1 cardiovascular trial data confirmed initial dosing strengths for Phase 2 trial in IH

- ✓ Matthew Plooster named Chairman of the Board of Directors
- ✓ Richard Pascoe appointed CEO
- ✓ Travis Mickle, Ph.D. transitioned to President
- ✓ Joshua Schafer appointed Chief Commercial Officer and EVP of Business Development
- ✓ Christopher Posner joined Board of Directors to serve as new independent director

## Corporate Initiatives

## Strong Balance Sheet to Support Value Creation

- ✓ Net revenue of \$2.9M includes revenue from arimoclomol EAP program in France
- ✓ Cash, cash equivalents and investments of \$107.4M as of Sept. 30, 2022
- ✓ Available capital expected to extend cash runway into 2026



## Focused on Creating Future Value in High Value Areas with Significant Unmet Needs; Solid Financial Foundation Creates Opportunities

Strategic Focus on Rare Diseases	<ul style="list-style-type: none"> <li>✓ Build a highly differentiated pipeline of development assets with multiple clinical and regulatory milestones</li> <li>✓ Focus on high-value areas with significant unmet needs in rare disease with potential to internally commercialize</li> </ul>
Arimoclomol: Treatment of Niemann-Pick disease type C (NPC)	<ul style="list-style-type: none"> <li>✓ NDA-stage drug candidate being developed for the treatment of NPC</li> <li>✓ “Capital efficient” financial structure; potential for positive cash flow; no shareholder dilution</li> <li>✓ NDA resubmission as early as Q3 2023; potential KemPharm commercial candidate</li> </ul>
KP1077: Treatment of Idiopathic Hypersomnia (IH) and Narcolepsy	<ul style="list-style-type: none"> <li>✓ High-value opportunity with significant unmet need</li> <li>✓ Initiated IH Phase 2 Trial in December 2022</li> <li>✓ Initiate Narcolepsy Phase 3 Trial post IH Phase 2 results</li> </ul>
Other Product Opportunities	<ul style="list-style-type: none"> <li>✓ Leverage prodrug platform to internally develop product candidates with significant potential value</li> <li>✓ Business development activities focused on complimentary clinical-stage rare disease assets</li> </ul>
AZSTARYS® License	<ul style="list-style-type: none"> <li>✓ Expanding launch of AZSTARYS provides ongoing revenue potential from royalties and milestones</li> </ul>
Strong Balance Sheet	<ul style="list-style-type: none"> <li>✓ Cash, cash equivalents and investments of <b>\$107.4M</b> as of September 30, 2022</li> <li>✓ Strong cash position supports development plan and other opportunities</li> <li>✓ Based on operating forecast, <b>cash runway expected to extend into 2026</b></li> </ul>



# **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

## Favorable Acquisition Terms

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



## High Upside Opportunity

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

## Early Access Programs

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

# About Niemann-Pick Disease Type C (NPC) <sup>1</sup>

Progressive Lysosomal  
Storage Disorder

Significant Unmet  
Need

Ultra Rare

- 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
- 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<sup>2</sup>
- No approved treatments exist in the U.S. for NPC
- NPC is estimated to occur in 1 in 100,000-120,000 live births
- Estimated 1,800 patients in the U.S. and Europe

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

# Arimoclomol – Innovative Product for a High Unmet Need

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's 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

*KemPharm 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 KemPharm

***KemPharm 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, KemPharm would currently be eligible to receive a Rare Pediatric Disease Priority Review Voucher
  - Last two vouchers sold in 2022 for \$110M each
  - 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

**Arimoclomol represents an opportunity for KemPharm to launch with a small, focused commercialization effort which can be foundation for future rare products, including KP1077**

- 1 Typically, ultra-rare disease commercial teams are less than 20 individuals which can be expanded as additional products are approved
- 2 Lower marketing spend since population is well defined and physicians are primarily located in treatment centers
- 3 Patient advocacy groups and relationships with treatment centers are also key drivers
- 4 Existing network of relationships with treatment centers and physicians already participating in early access programs in the U.S. and E.U.
- 5 Partnerships/licensing opportunities may be available in other markets (Japan, China, others)



# **SDX Product Candidate: KP1077**

**For the Treatment of Idiopathic Hypersomnia (IH)**



# KP1077 – Product Candidate Overview

*KemPharm 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

## Regulatory & IP Advantages

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



## 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

## 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<sup>1</sup>
- ✓ ~37,000 diagnosed IH patients actively seeking treatment<sup>2</sup>
- ✓ 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)

## Ultra 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)<sup>3</sup>

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 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

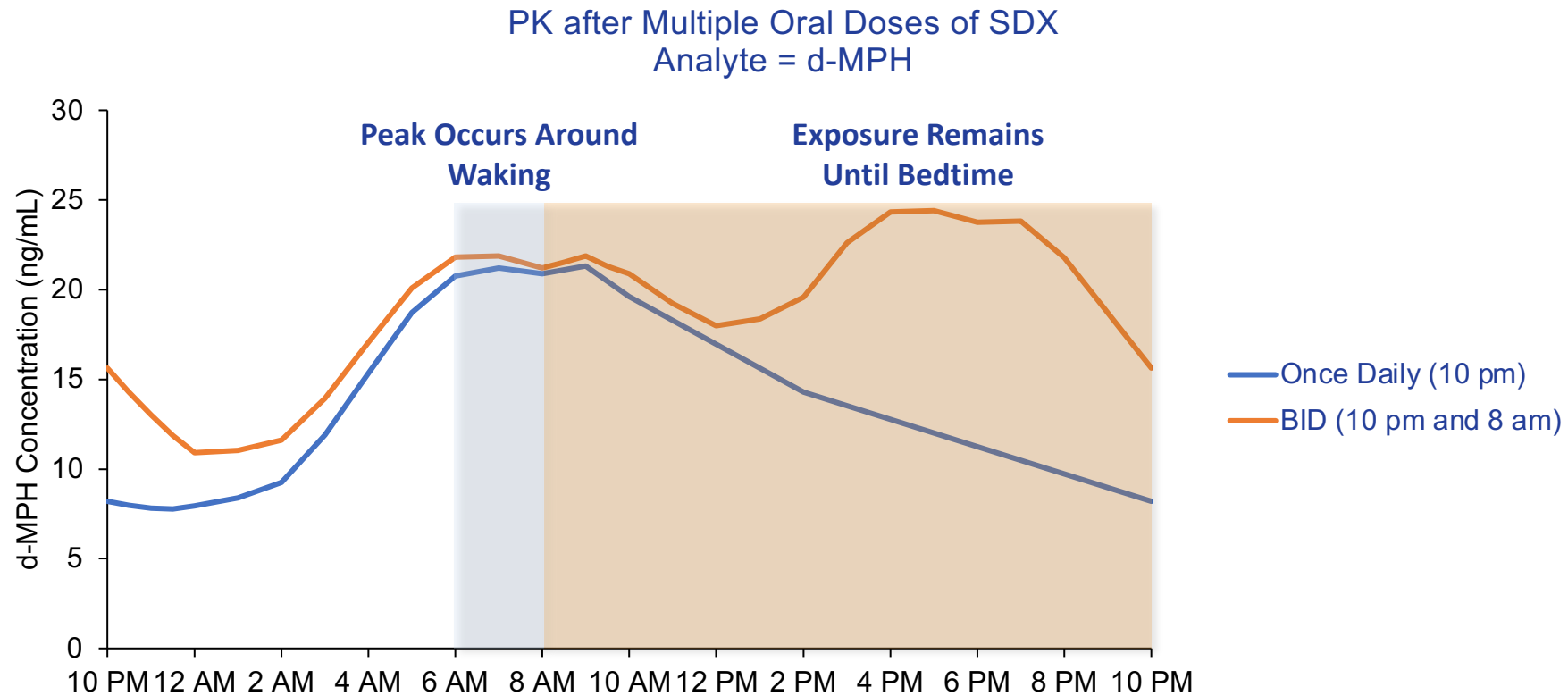
- *Brain fog in IH is so debilitating that current, tolerable stimulant treatment doses are inadequate:*
  - The ability to dose higher with fewer negative side-effects, including those associated with blood pressure (BP) and heart rate (HR), compared to current off-label treatments have the potential 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)<sup>1</sup>*
  - 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



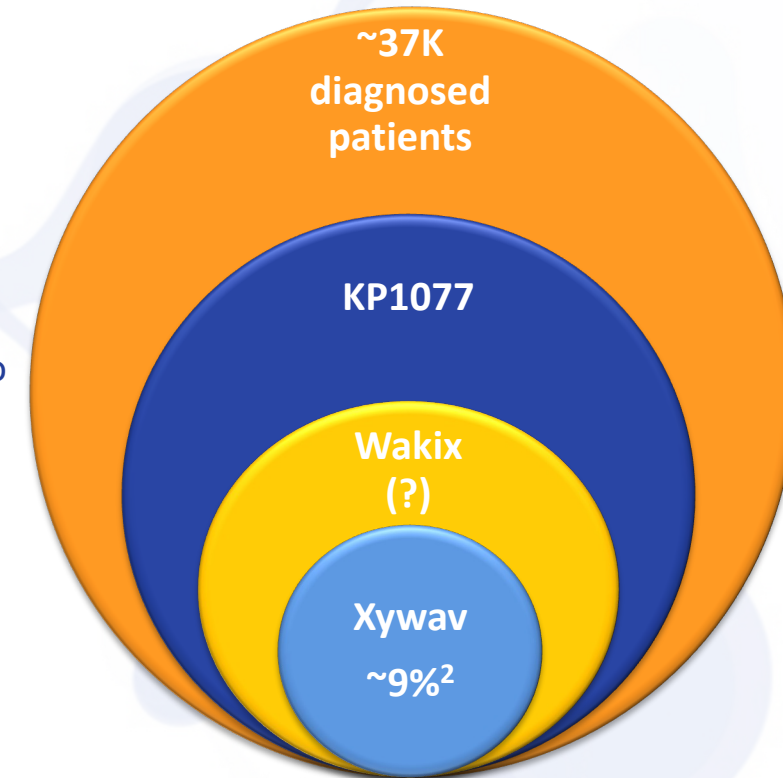
Plasma concentrations were estimated based on data collected in study KP879.101

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 Based on Potential Clinical Differentiation and Combination Use

- It is estimated that ~37K patients are currently diagnosed with IH and actively seeking treatment<sup>1</sup>
- 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
  - Assuming an average price of ~\$94K per patient per year, IH patient share for Xywav by 2025 is expected to be ~3,200 patients (~9% of diagnosed patients)<sup>2</sup>
- Potential factors that may result in higher adoption of KP1077, compared to Xywav or Wakix®:
  - **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<sup>3</sup>
  - **Xywav promotion and disease awareness:** may result in expansion of diagnosed patient population (e.g., Jazz Pharmaceuticals and Hypersomnia Foundation launched a campaign to increase understanding and awareness about idiopathic hypersomnia in March 2021)<sup>2</sup>
  - **Wakix barriers to uptake:** DDI, especially with antidepressants and antihistamines

## Illustrative Market Share based on Potential Differentiation



Sources: (1) <https://www.sleepcountshcp.com/what-is-idiopathic-hypersomnia>  
(2) <https://investor.jazzpharma.com/investors/events-presentations>

(3) <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

*KP1077 Represents a Potential “Portfolio in a Pill” Opportunity*

## Idiopathic Hypersomnia

- Lead KP1077 indication
- Investigational New Drug (IND) application cleared by FDA
- Initiation of Phase 2 clinical trial anticipated prior to year-end 2022
- Interim data from Phase 2 clinical trial expected by mid-year 2023
- Top-line data available by EOY 2023

## Narcolepsy

- Second KP1077 indication would allow KemPharm to address two rare sleep indications that are underserved by currently available medications
- Initiate narcolepsy Phase 3 Trial post IH Phase 2 results
  - Leverage key data points from IH program to expedite narcolepsy development



**AZSTARYS®**

**d-Methylphenidate Prodrug Product  
for the Treatment of ADHD**



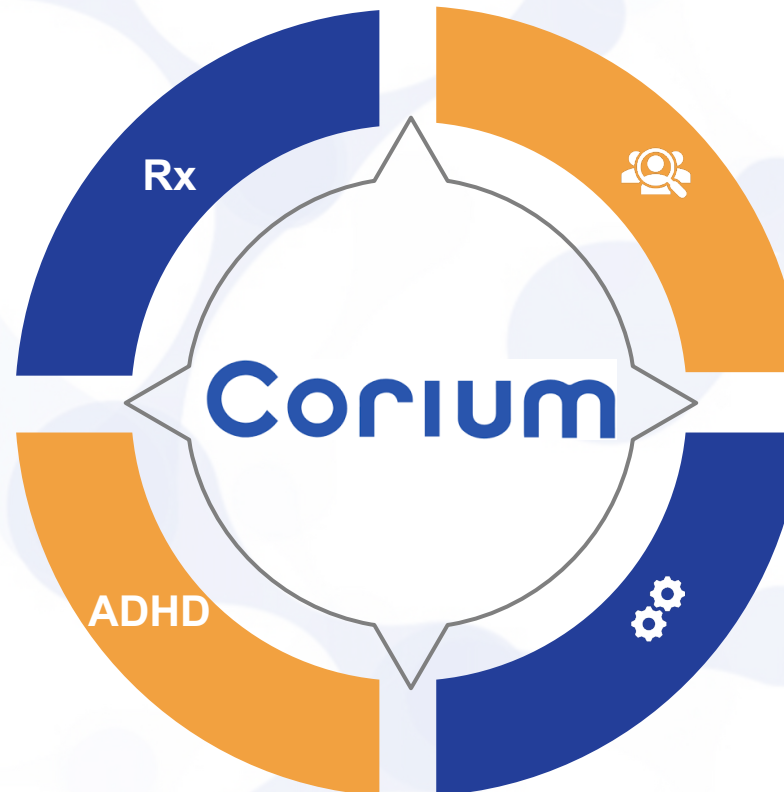
# Corium, Inc. – AZSTARYS® Commercialization

## 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 and 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



# **Financial Update and Upcoming Milestones**



# Financial Position is a Source of Strength

## Q3 2022 Income Statement Details:

- Net revenue of \$2.9M, primarily from the arimoclomol EAP program in France
- Q3 2022 net loss attributable to common stockholders of (\$6.6M), or (\$0.19) per basic and diluted share, driven primarily by R&D expense of \$5.4M, and general and administrative expense of \$4.0M
  - Partially offset by net revenues of \$2.9 million

## Balance Sheet Details as of Sept. 30, 2022:

- Cash, cash equivalents and investments were \$107.4M, a decrease of \$7.1M compared to Q2 2022
  - Driven in part by increased third-party research and development costs related to the KP1077 clinical trial program, the arimoclomol program, other expenses, as well as investment of working capital related to the collection of accounts receivable due from French EAP reimbursements
- Available cash, cash equivalents and investments expected to extend cash runway into 2026



# KemPharm: Multiple Growth Catalysts

- ✓ Potential to re-file NDA as early as Q3 2023
- ✓ Anticipated ongoing quarterly revenue from EAP program in France

**Arimoclomol**

**KP1077**

- ✓ Phase 2 trial in IH initiated
- ✓ Interim Phase 2 IH efficacy and safety data expected as early as Q3 2023
- ✓ Phase 3 trial in narcolepsy to initiate following IH Phase 2 trial results

- ✓ Potential to realize sales milestone and royalty revenue from AZSTARYS, providing further capital flexibility and extending operating cash runway

**AZSTARYS®**

**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





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**Management Presentation**

**January 2023**