KemPharm INC

Management Presentation

October 2022

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KEMPHARM VALUE PROPOSITION

Innovative pharma company with a proven regulatory track record targeting rare CNS, neurodegenerative and lysosomal storage diseases

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 beyond 2025

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

Strategic Focus on CNS/Rare Disease	✓ Build a highly differentiated pipeline of development assets with multiple clinical and regulatory milestones ✓ Focus on high-value areas with significant unmet needs in CNS/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 ✓ Potential to re-file NDA as early as Q1 2023; potential KemPharm commercial candidate
KP1077: Treatment of Idiopathic Hypersomnia (IH) and Narcolepsy	✓ High-value opportunity with significant unmet need ✓ Initiate IH Phase 2 Trial by the end of 2022 ✓ Initiate Narcolepsy Phase 3 Trial post IH Phase 2 results
Other SDX Product Opportunities	✓ Versatility of the SDX family of product candidates with significant potential value; "pipeline in a pill" ✓ Multiple potential indications with initial focus in sleep disorders
AZSTARYS® License	✓ Expanding launch of AZSTARYS provides ongoing revenue potential from royalties and milestones
Strong Balance Sheet	✓ Cash and equivalents of **$114.5M** as of June 30, 2022 ✓ Strong cash position supports development plan and other opportunities ✓ Combined with forecasted revenues, **cash runway beyond 2025**

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) [1]

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 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 Appears Straightforward

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

CRL Analysis

- Current plan to address the major issues related to the CRL appear addressable
  - ✓ Primary instrument (NPCCSS) work has concluded
  - ✓ Additional analyses have been conducted
  - ✓ Confirmatory evidence has been significantly augmented

Regulatory Outlook

- We believe there is a viable pathway that could enable a successful NDA resubmission and subsequent approval for arimoclomol in NPC
  - ✓ Path may include additional non-clinical or clinical studies (long-term or efficacy trials unlikely)
  - ✓ Federal Dispute Resolution Request (FDRR) may be utilized, if necessary
  - ✓ An advisory committee (ad com) may also be utilized either by FDA or KemPharm

KemPharm expects to resubmit the NDA for arimoclomol in NPC as early as Q1 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 already generating revenue through the French EAP system
  - 34 patients in the French EAP as of Mar 31, 2022
  - 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**
  - As of Mar 31, 2022: 67 patients in U.S., 41 patients Germany, 34 patients in France, and 9 patients in other countries (Denmark, Switzerland and UK)

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

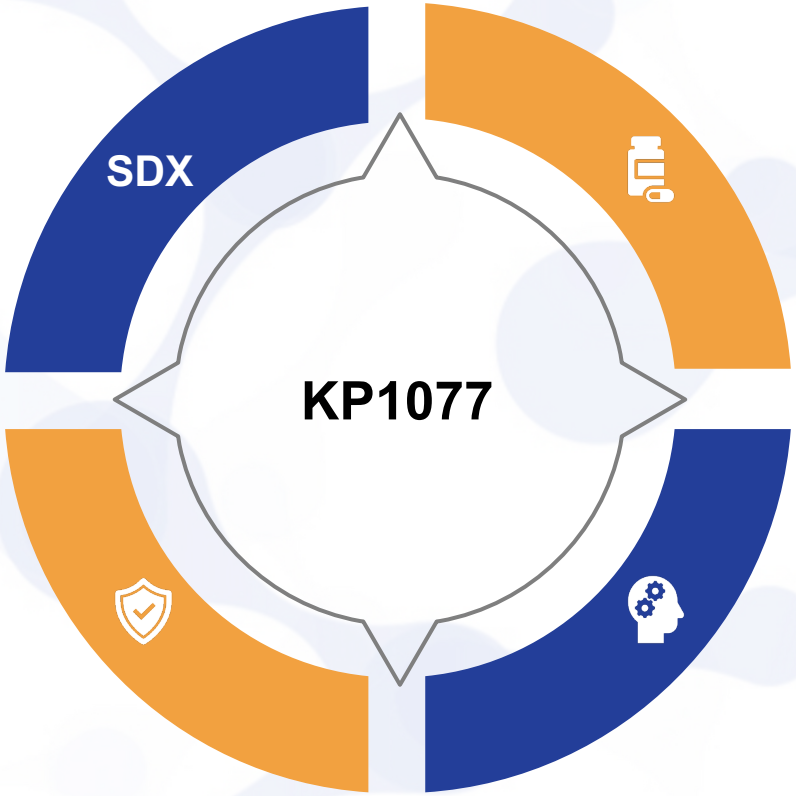
KemPharm is advancing KP1077 as a potential therapeutic treatment for Idiopathic Hypersomnia (IH)

Serdexmethylphenidate

- ✓100% SDX with multiple dosing options
- ✓SDX has already been 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[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
  - Long and unrefreshing naps
  - 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)[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

KP1077: Addressing Cardiovascular Stimulant Comorbidities

Many comorbidities and patient demographics complicate treatment, including cardiovascular issues

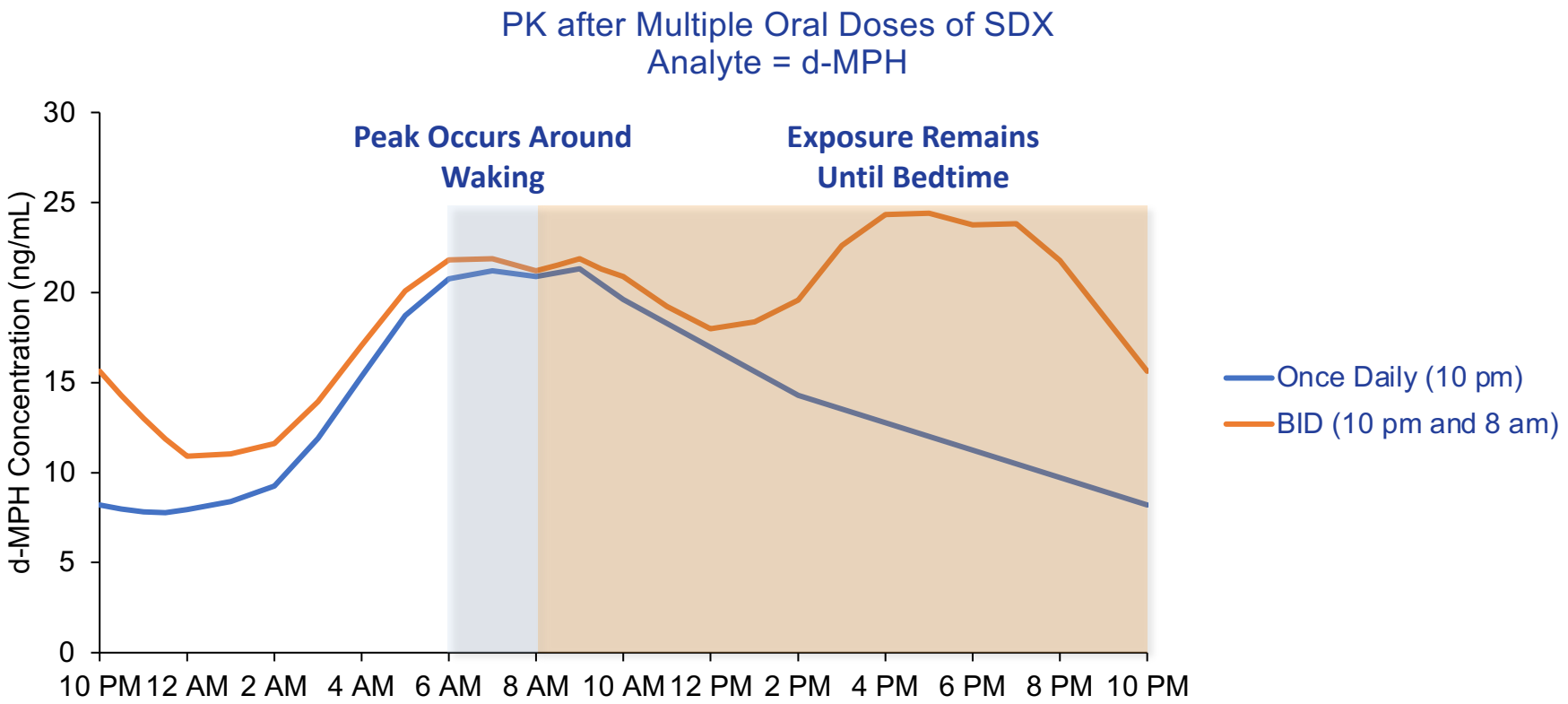
- *Brain fog in IH is so debilitating that current, tolerable stimulant treatment doses are inadequate*:
  - The ability to dose higher with fewer negative side-effects, including those associated with blood pressure (BP) and heart rate (HR), compared to current off-label treatments have the potential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

- Planned Phase 2 trial doses should be well tolerated and potentially address the most problematic symptoms of IH
- Phase 2 trial expected to start Q4 2022

(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



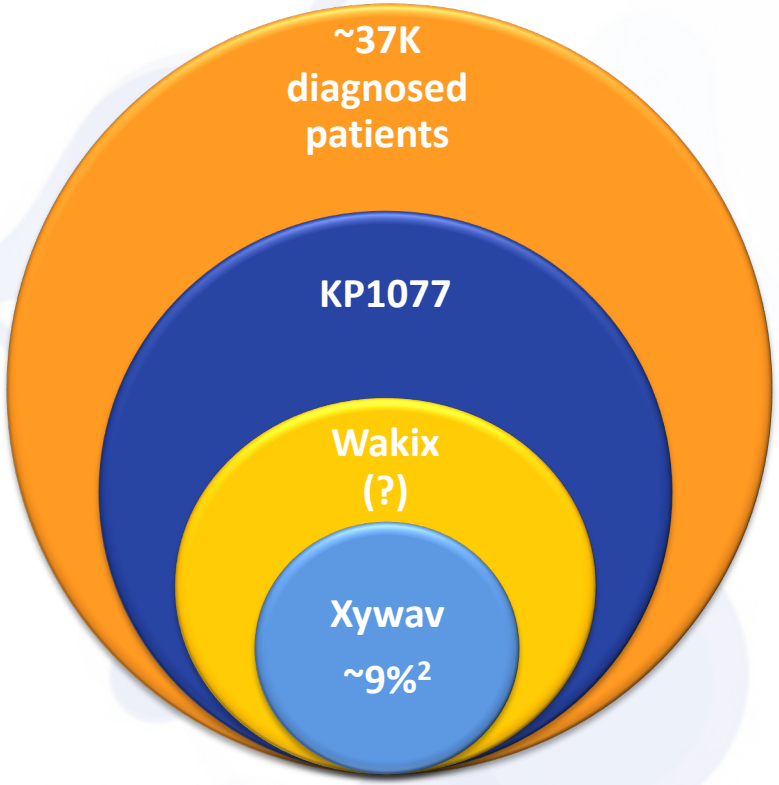
Plasma concentrations were estimated based on data collected in study KP879.101

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[1]
- 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)[2]
- 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[3]
  - **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)[2]
  - **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/

AZSTARYS®

d-Methylphenidate Prodrug Product for the Treatment of ADHD

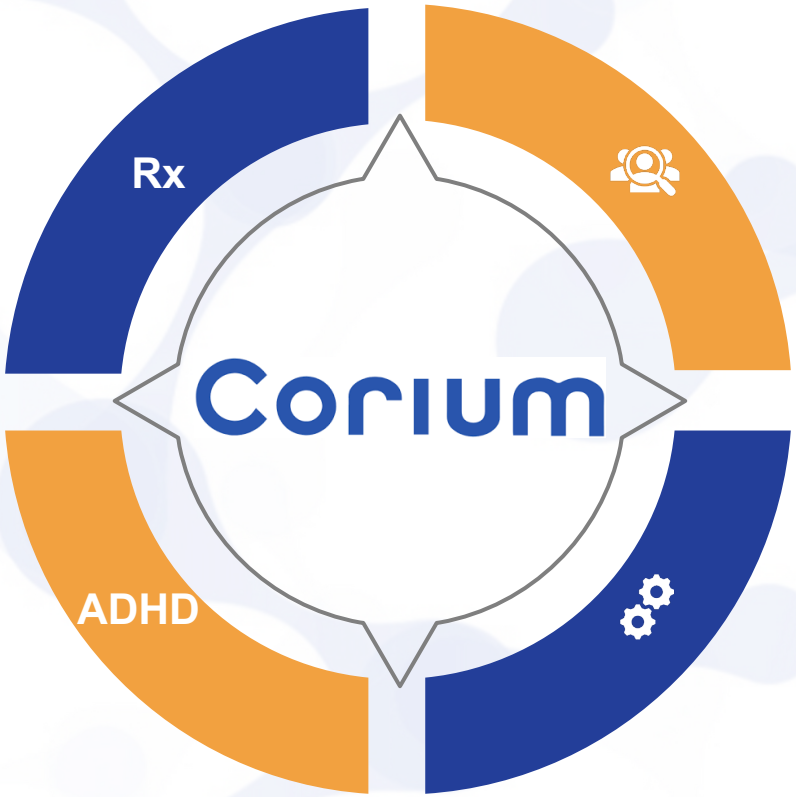
Corium, Inc. – AZSTARYS® Commercialization

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

Q2 2022 Income Statement Details:

- Net revenue of $1.3M, derived from arimoclomol product sales under the French EAP, consulting services fees and royalties
- Q2 2022 net loss attributable to common stockholders of ($24.0M), or ($0.70) per basic and diluted share, driven primarily by one-time non-cash expense related to the arimoclomol asset acquisition of $17.7M
- Adjusted net loss excluding the arimoclomol asset acquisition expense is ($6.4M), or ($0.19) per basic and diluted share

Balance Sheet Details as of June 30, 2022:

- Cash, cash equivalents and investments were $114.5M, a decrease of $4.6M compared to Q1 2022
- Line of credit for the arimoclomol acquisition expected to be serviced by the cashflow from French EAP
- Available cash, cash equivalents and investments extends cash runway beyond 2025

Upcoming Clinical and Regulatory Milestones Create Potential Near-Term Value

Milestone	Q2 2022	Q3 2022	Q4 2022	Q1 2023	Q2 2023	Q3 2023	Q4 2023
Arimoclomol							
Re-file NDA for NPC				■			
KP1077 for IH							
IND filing/may proceed	✓						
Phase 1 CV differentiation trial	✓	✓					
Phase 2 trial		■	■	■	■	■	■
KP1077 for Narcolepsy							
Type B meeting with FDA			■				
IND filing			■	■			
Phase 2/3 trial initiation				■	■	■	■

*Note: **Blue** box denotes activity timeframe*

KemPharm: Strong Foundation for the Next Phase of Growth

- ✓ NDA-stage asset expands pipeline targeting rare orphan CNS diseases
- ✓ "Capital efficient" deal structure w/potential for positive cash flow, and no shareholder dilution
- ✓ Potential to re-file NDA as early as Q1 2023

Arimoclomol Acquisition

KP1077 Development Program

- ✓ KP1077 IND for IH submitted to FDA
- ✓ Phase 2 trial initiation in IH by the end of 2022
- ✓ Phase 1 clinical trial results confirmed cardiovascular safety risk of SDX and could allow for higher, more effective dosing

AZSTARYS® National Launch

Strong Balance Sheet to Support Value Creation

- ✓ Full national team in place; ~175 reps
- ✓ ~145M+ covered commercial lives; preferred status for 35 million of those covered lives
- ✓ Expanded launch of AZSTARYS supports revenue potential from royalties and milestones

- ✓ Cash, cash equivalents and investments of **$114.5M** as of June 30, 2022
- ✓ Solid balance sheet supports development efforts and other pipeline expansion activities
- ✓ Available capital extends cash runway beyond 2025

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