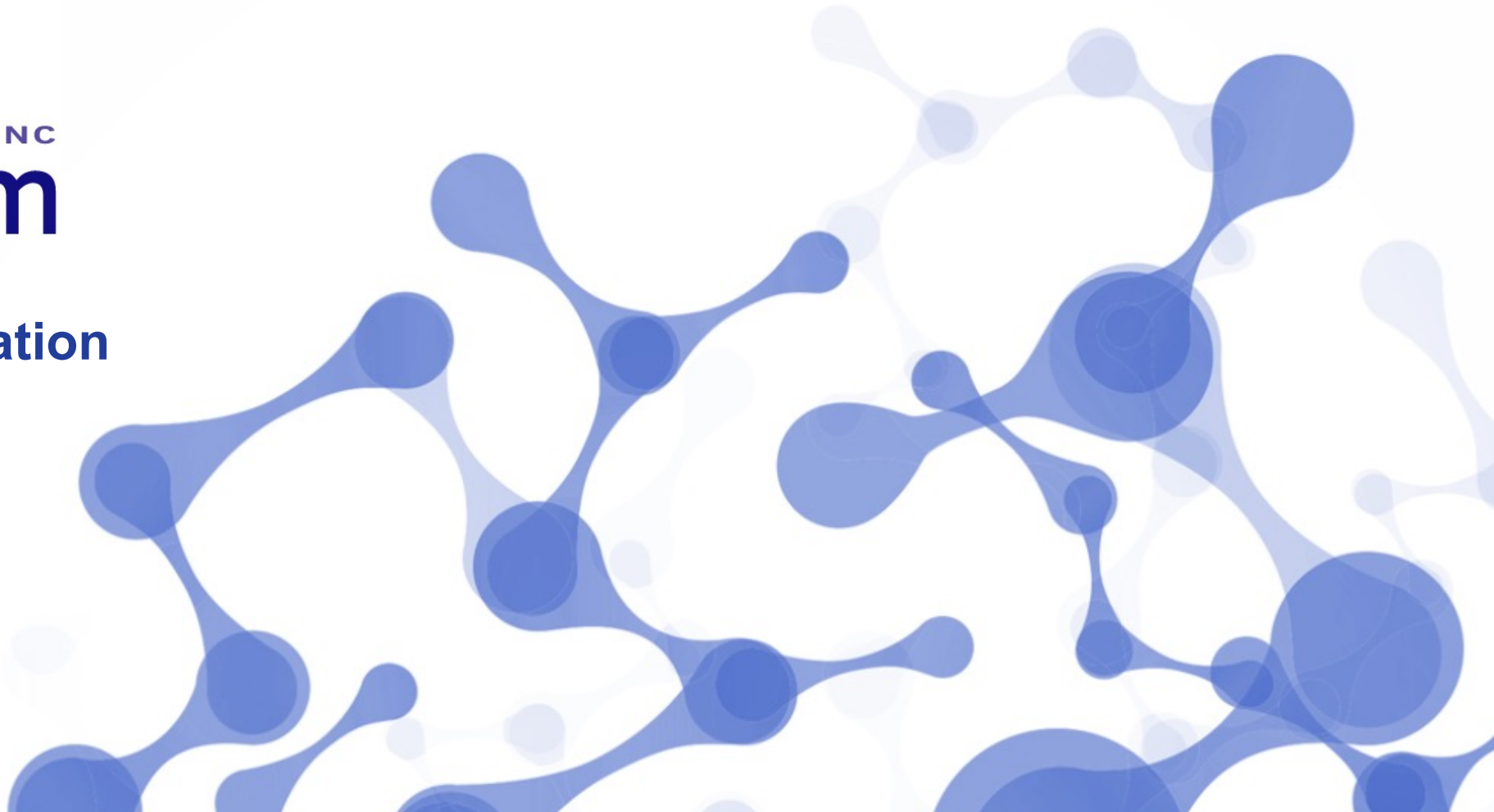
KemPharm INC

Management Presentation

May 2022



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KEMPHARM VALUE PROPOSITION

Innovative pharma company developing and commercializing novel treatments for rare CNS, neurodegenerative and lysosomal storage diseases

Diverse product portfolio combining clinical-stage pipeline with revenue-generating NDA-stage and commercial assets

Two FDA approved and partnered medications, AZSTARYS® and APADAZ®, validate approach and regulatory expertise

Experienced Management Team in Corporate and Drug Development

Travis C. Mickle, PhD President and CEO	R. LaDuane Clifton, CPA CFO	Sven Guenther, PhD EVP R&D
azstarys serdexmethylphenidate and dexmethylphenidate 26.1/5.2mg • 39.2/7.8mg • 52.3/10.4mg capsules apadaz benzhydrocodone and acetaminophen CII ONCE-DAILY Vyvanse CII (lisdexamfetamine dimesylate) 10 • 20 • 30 • 40 • 50 • 60 • 70 mg capsules 10 • 20 • 30 • 40 • 50 • 60 mg chewable tablets	Aetna LGL GROUP KPMG	azstarys serdexmethylphenidate and dexmethylphenidate 26.1/5.2mg • 39.2/7.8mg • 52.3/10.4mg capsules apadaz benzhydrocodone and acetaminophen CII ONCE-DAILY Vyvanse CII (lisdexamfetamine dimesylate) 10 • 20 • 30 • 40 • 50 • 60 • 70 mg capsules 10 • 20 • 30 • 40 • 50 • 60 mg chewable tablets

Collective Team Experience:

Shire, New River Pharmaceuticals, Salix Pharmaceuticals, Inc., Cephalon, Amarin, Valeant, Synchrony Healthcare Communications, PanPacific Pharmaceuticals, Inc., Chiron, Abbott, sigma-tau Pharmaceuticals, Inc. Rare dedication

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, revenue-generating 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 soon as Q1 2023; potential KemPharm commercial candidate
KP1077: Treatment of Idiopathic Hypersomnia (IH)	✓ High-value opportunity with significant unmet need ✓ Represents potential for meaningful near-term value with multiple clinical milestones
Other SDX Product Opportunities	✓ Versatility of the SDX family of product candidates with significant 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 **$119.1M** as of Mar 31, 2022 ✓ Strong cash position supports development plan and other opportunities ✓ Combined with forecasted revenues, **cash runway extends to 2025 and beyond**

Full Pipeline of Product Candidates with Substantial Milestones in 2022 and Beyond

Arimoclomol

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

Arimoclomol Acquisition - Expanding Pipeline Targeting Rare Diseases

Definitive agreement with Orphazyme A/S to acquire substantially all assets, including arimoclomol

- This acquisition is a significant expansion of KemPharm's development pipeline targeting rare diseases; allows KemPharm to leverage prior experience with challenging regulatory situations
  - Aligns with strategy to build value through the development and commercialization of novel treatments for rare diseases
- Arimoclomol is an NDA-stage, revenue-generating investigational drug candidate being developed for the treatment of Niemann-Pick disease type C (NPC), an ultra-rare progressive, disabling and fatal lysosomal storage disorder
  - Arimoclomol is currently available to NPC patients in the U.S., France, Germany and other European countries under early access programs (EAPs)
  - 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
  - USD $12.8M cash payment for substantially all assets and operations of Orphazyme; French EAP is expected to generate revenue in excess of USD $12M in FY 2022

Definitive Agreement – Summary of Terms

- KemPharm will purchase substantially all of the assets and operations of Orphazyme, including arimoclomol, with a cash payment of USD $12.8 million
  - Cash payment will be financed with a revolving line of credit secured by KemPharm's balance sheet
  - Ongoing cashflow from early access programs will service the debt
- KemPharm will assume estimated reserve liabilities of USD $5.2 million which is an estimated future rebate due to the French regulatory authorities based on revenue generated from the EAP in France
  - **EAP for arimoclomol in France expected to generate revenue in excess of $12 million (USD) in FY 2022 based upon the actual Q1 2022 enrollment**
  - EAP expected to remain in place until arimoclomol becomes available commercially in France
- KemPharm intends to retain the majority of Orphazyme's current employees and continue operations through a new subsidiary in Denmark
- Transaction expected to close on or before June 1, 2022, subject to final approval by Orphazyme's creditors and the Danish bankruptcy court

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

- **Ultra-rare 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
- Most cases are detected during childhood and progress to cause life-threatening complications
  - 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
  - NPC is estimated to occur in 1 in 100,000-120,000 live births
  - **The mean age of death in NPC patients is 13 years[2]**
  - Estimated 1,800 patients in the U.S. and Europe
- **No approved treatments exist in the U.S. for NPC**
  - In Europe, there is only one treatment available, migulstat

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 improved lysosomal and cellular function with arimoclomol treatment
- Studied in ten Phase 1, four Phase 2, and three Phase 2/3 trials in various rare diseases
  - Positive efficacy results from NPC trial (NPC-002)
  - Positive results from a Phase 2 trial in Gaucher's Disease (GD), a related lysosomal storage disorder
  - Safety data has been collected from more than 500 individuals for up to 5 years of treatment with no significant safety findings identified to date
- Received **Orphan Drug Designation** for NPC in the U.S. and EU; and **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 the FDA***
  - Eligible to receive New Chemical Entity (NCE) and Orphan Drug Exclusivity
  - Patents and patent applications, if issued, could extend exclusivity through 2040

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

- Orphazyme received a Complete Response Letter (CRL) from the FDA on Jun 17, 2021, regarding their NDA for arimoclomol for the treatment of NPC
  - The FDA identified three issues:
    1) Additional evidence needed to substantiate validity of the primary endpoint used in the single efficacy trial
    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 was held on Oct 13, 2021:
  - *FDA agreed* to allow a reanalysis of the primary endpoint 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 discussion of how best to handle missing data; no consensus was obtained at the meeting
  - Additional confirmatory evidence was provided to the FDA at the meeting; further confirmation from FDA as to the impact of those studies will be important

FDA Agreed with Proposed Plan to Strengthen Primary Endpoint and Analysis

1) Conduct a new analysis of the original 5-domain NPCCSS primary endpoint by removing the cognition domain (ambulation, swallowing, speech and fine motor skills remain) to form a new 4-domain NPCCSS endpoint
   - ✓ FDA confirmed that this is acceptable
2) Conduct a qualitative study to assess the validity and robustness of the swallowing domain, the scoring used in the trial and the clinical relevance
   - ✓ FDA confirmed this is acceptable and has reviewed and provided comments on the protocol
   - ✓ Study complete and should address the FDA's issue
3) Use the log ratio transformed analysis to address the FDA's concerns regarding missing data
   - No direct commitment from the FDA
   - Still needs discussed in detail and alternatives should be explored

Ultimately, using the prespecified analysis in the SAP with the original 5-domain NPCCSS endpoint met statistical significance and study *NPC-002 WAS SUCCESSFUL*.

- Agency's methodology was post-hoc and **NOT** prespecified; other statistical methods may be more appropriate **including the prespecified analysis originally agreed upon with the FDA**
- Missing data discussion is a common issue for sponsors with the FDA, especially in rare diseases

Modification of Primary Endpoint with FDA Agreement Demonstrates Improved Statistical Significance and Treatment Effect

Analysis	Full Population[1]		Migulstat Subgroup[1]	
	Treatment Effect[2]	p-value	Treatment Effect[2]	p-value
Prespecified 5-domain NPCCSS	-1.4	0.0456	-2.06	0.0060
Prespecified 4-domain NPCCSS	-1.54	0.0193	ND	ND
FDA post-hoc 5-domain NPCCSS	-1.24	0.1093	-2.23	0.0031
FDA post-hoc 4-domain NPCCSS	-1.33	0.0602	-2.23	0.0018
Prespecified 4-domain NPCCSS and potential swallowing rescore[3]	-1.70	**0.0150**	ND	ND
FDA Post-hoc 4-domain NPCCSS and potential swallowing rescore[3]	-1.48	**0.0470**	ND	ND

1. Analysis conducted as full population and migulstat subgroup. Miglustat was used as the Standard of Care treatment in roughly 80% of all patients including placebo. 5-domain NPCCSS is based on a scale from 0 to 25 broken up in five groups of 0 to 5 with zero considered normal and 5 the worst symptom for each domain. Domains include ambulation, speech, swallowing, fine motor skills and cognition. The 4-domain analysis removes cognition for a total potential score of 20.
2. Treatment effect as measured as a change from baseline. A decrease in score is considered an improvement and a change in score of one (or negative one) is considered clinically meaningful.
3. Analysis discussed with FDA at Type A meeting but not agreed upon as swallowing analysis and potential rescoring was not complete.

ND designates not determined

Bolstering Confirmatory Evidence Addresses Another Key Issue Raised in CRL

- **Since the CRL and Type A meeting, additional data has already been generated**
  - Numerous studies and additional analysis has been conducted to address this issue
  - Primary focus was to confirm or elucidate the mechanism of action, the potential beneficial effect of migulstat, and to further support the clinical data
  - Some of this new data includes:
    - ✓ Biomarkers
    - ✓ Results from open-label extension arms
    - ✓ Data from expanded access programs
    - ✓ In vitro studies
    - ✓ In vivo models of NPC
- **Totality of the evidence provided appears to support the clinical outcome**
  - Now that there is a sizable amount of new data available, FDA input will be sought to verify it is sufficient to address their issue

Path to Resubmission and Approval Appears Straightforward

- Current plan to address the major issues related to the CRL appear addressable
  - ✓ Primary endpoint work has concluded
  - ✓ Additional analyses have been conducted
  - ✓ Confirmatory evidence has been significantly augmented
- KemPharm has significant experience with challenging regulatory situations, including two FDA product approvals that followed initial CRLs and dealing with the FDA and statistical issues that can occur in clinical trials
  - Based on our experience and assessment of regulatory situation, 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, though it is not expected that any long-term or efficacy trials would be needed
    - Federal Dispute Resolution Request (FDRR) may be utilized, if necessary
    - An advisory committee (ad com) may also be required by FDA after resubmission
- **We expect to resubmit the NDA for arimoclomol in NPC <u>as early as Q1 2023</u>**

Arimoclomol Market Opportunity Is Compelling, Even With Ultra-Rare Status

- Upon approval, KemPharm would currently be eligible to receive a Rare Pediatric Disease Priority Review Voucher
  - Last voucher sold for $110M, which was within the typical range of approx. $100M/voucher
  - Program could eventually end, making these vouchers more scarce
- 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
  - Program typically remains in place while therapy is moving towards a marketing application and potential French and/or European approval
- **Global EAP programs represent the potential first adopters of arimoclomol post-approval**
  - Currently there are 151 global participants, with enrollment rising
  - 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 is an NDA-stage, revenue-generating product upon which we intend to build commercial capabilities that fit with the goal of allowing KemPharm to create and retain value for the benefit of shareholders
- **Arimoclomol represents an opportunity for KemPharm to launch with a small, focused commercialization effort that can be foundation for future rare products, including KP1077**
  - Typically, ultra-rare disease commercial teams are less than 20 individuals which can be expanded as additional products are approved
  - Lower marketing spend since population is well defined and physicians are usually primarily in treatment centers
  - Patient advocacy groups and relationships with treatment centers are also key drivers
  - Establishes a commercial platform that can be leveraged with other products, including KP1077
- **Arimoclomol represents a global market opportunity**
  - Current patients enrolled in the EAPs in U.S., France, Germany, and other European countries expected to transition directly to commercial once approved within each market
  - Partnerships/licensing opportunities may be available in other markets (Japan, China, others)

SDX Product Candidate: KP1077

For the Treatment of Idiopathic Hypersomnia (IH)

Idiopathic Hypersomnia (IH)

- 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 are highly debilitating – **IH can be more debilitating than narcolepsy**
  - Chronic daytime sleepiness
  - Long and unrefreshing naps
  - Extreme difficulty waking (sleep inertia and/or sleep drunkenness)
  - Severe brain fog
  - Some experience excessively long sleep times (~25% of patients "long sleepers", >10hrs)
- 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
  - Patient survey data indicates that current medication effectiveness was poorly rated at 5.4/10[(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 Product Candidate Overview

- 100% Serdexmethylphenidate (SDX) product with multiple dosing options depending on patient needs
  - Dosed either QD (1x daily at bedtime) or BID (2x daily at bedtime and upon waking)
- Features and benefits already demonstrated:
  - **SDX has already been designated C-IV by DEA**
  - No DDI potential with hormonal contraceptives and antidepressants
- Potential additional features and benefits to be studied:
  - **Greater tolerability** could allow for higher, more effective dosing (i.e. greater efficacy)
  - Dosing regimen addresses the two primary issues associated with IH
    - Night-time dosing addresses sleep inertia (waking)
    - Morning dosing addresses daytime brain fog; considered most problematic symptom of IH
  - **Lessened effect on heart rate and blood pressure** vs. other MPH products
- Eligible for Fast-Track, Orphan Drug and Breakthrough Therapy designations
- No generic equivalent and not substitutable; **solid IP through 2037** and potentially beyond

If Differentiated, KP1077 Could Gain Significant Share if Priced Between Provigil® and Xywav®/Wakix®

Brand Name Active Ingredient	Sponsor	DEA Schedule	Features	Annual Cost
Xywav (mixed oxybate salts)	Jazz	C-III	• Approved for IH; centrally acting depressant • Dosed twice at night; once before bed and another 4 hrs later • 75% of patients in Xywav IH trial maintained or added stimulant treatment	**Highest dose: $187,000/year**
Provigil/Nuvigil® (modafinil/armodafinil)*	Teva	C-IV	• Approved for treatment of EDS associated with narcolepsy • Numerous drug-drug interactions including with hormonal contraception and antidepressants • Serious adverse events include Stevens-Johnson Syndrome, angioedema, anaphylaxis and multi-organ hypersensitivity	**Provigil: $24,000/year**
Various IR/ER methylphenidate products*	Various brands and generics	C-II	• Ritalin® indicated for the treatment of narcolepsy • Ritalin daily dose not to exceed 60 mg • Elevated blood pressure and heart rate; serious cardiovascular effects may also occur	**Varies: ~$4,000-$5,000/year**
Wakix (pitolisant)*	Harmony Biosciences	Not Scheduled	• Approved for treatment of EDS or cataplexy in narcolepsy • Significant drug-drug interactions including antidepressants and antihistamines • Contraindicated in severe hepatic impairment • QT interval prolongation	**Highest dose: $157,000/year**

Note: Information on this slide was located within each respective package insert; products potentially used off-label for IH are indicated with an *

KP1077 Value Proposition: Addressing Key Unmet Needs

- **Idiopathic hypersomnia can be more debilitating than narcolepsy**
  - *Sleep inertia/waking*: nightly dosing provides increased d-methylphenidate (d-MPH) concentrations upon waking
  - *"Brain fog":* morning dosing provides long-lasting d-MPH concentrations throughout the entire day
  - The PK profile of KP1077 dosed BID before bed and upon waking provides increased d-MPH concentrations early in the morning upon waking, increased concentrations in the afternoon and a steady concentration throughout the entire waking day
- **There are no approved stimulant therapies for the treatment of IH**
  - *No current therapy adequately addresses sleep inertia and brain fog:* KP1077 can address both AND as already suggested by recent trial results with SDX, at higher concentrations of d-MPH compared to other MPH and stimulant products. This is due to the slow release of d-MPH and lack of significant peaks in concentrations (C_{max}) post-administration. Higher, more tolerable doses of d-MPH may be more efficacious especially in treating brain fog.
  - Patient data shows that current treatments are not effective at controlling symptoms (see Slide 8)
  - Only one other product, Wakix® (pitolisant), is under development in IH

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 recently initiated to assess cardiovascular safety of SDX compared to immediate-release and long-acting formulations of Ritalin®**
  - SDX's pharmacokinetic release profile of d-MPH may avoid adverse events associated with large and rapid exposure fluctuations observed with other stimulant-based therapies
  - Topline results expected as early as Q3 2022

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

MOA[1] and Trial Data Suggest KP1077 is Well Positioned to Demonstrate Efficacy

- **Ritalin and Ritalin SR (racemic methylphenidate) are indicated to treat Narcolepsy[2]**
  - *Methylphenidate-based products have demonstrated some efficacy in treating excessive daytime sleepiness associated with Narcolepsy*

- **Phase 1 trial results suggest SDX at higher-doses can produce the desired effects of wakefulness and a feeling of being energized in subjects with a history of high-dose stimulant use**
  - *Additional effects of hypervigilance and insomnia were also indicative of the potential to address excessive sleepiness*
  - *Trial data collected during AZSTARYS development provides further evidence of potential effects and pharmacokinetics*

Drowsiness/Alertness VAS[a]

(bipolar scale: 0 to 100)

Baseline Score Range:	**37 – 50**
Peak Score Range:	**67 - 89**

Energized VAS[b]

(unipolar scale: 0 to 100)

Baseline Score Range:	**15 - 20**
Peak Score Range:	**61 - 82**

Notes:

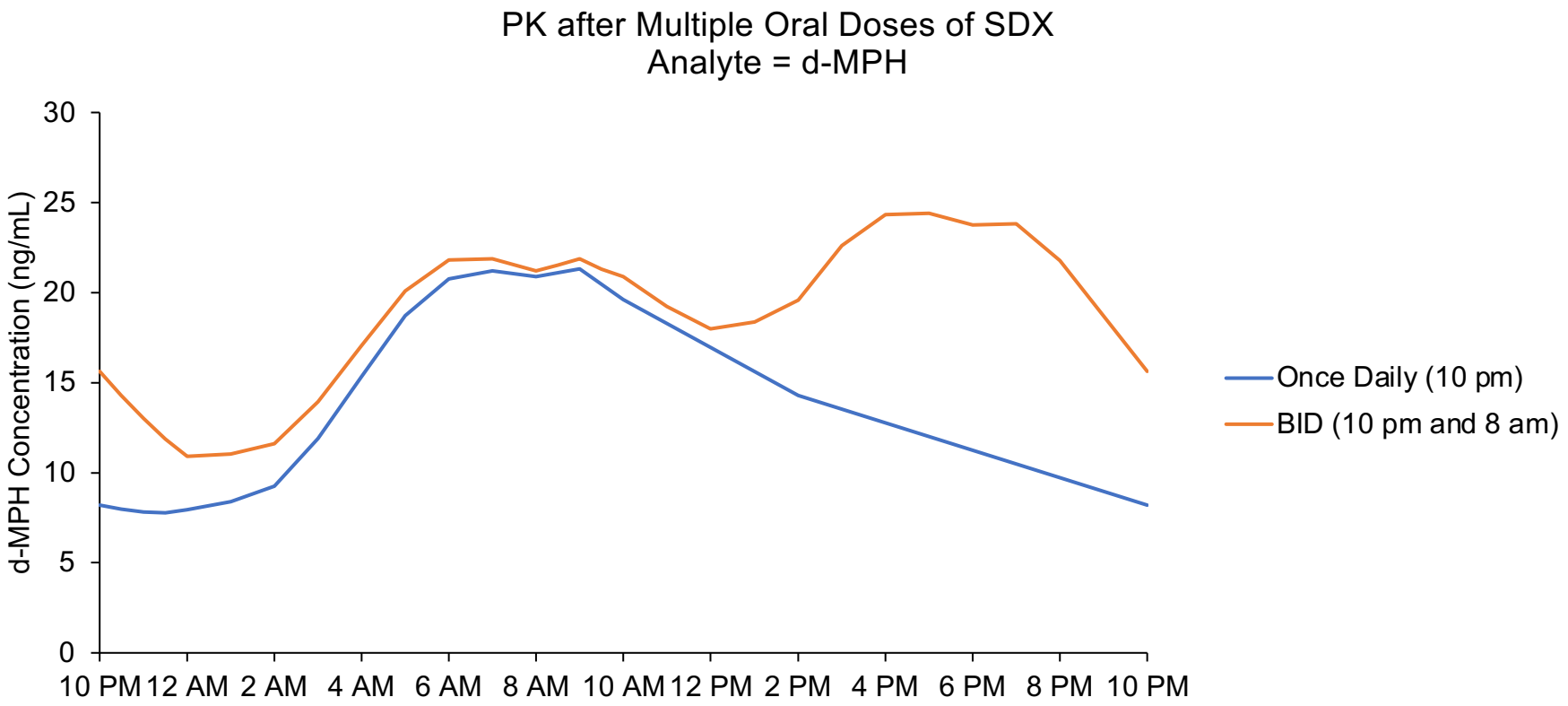
(a) Drowsiness/Alertness Visual Analogue Scale is an at-the-moment bipolar scale where a score of 50 is neither drowsy or alert, a score of 0 is Strong Drowsiness and a score of 100 is strong Alertness

(b) Energized Visual Analogue Scale (VAS) is an at-the-moment unipolar scale measuring the feeling of excess energy where a score of 0 is “definitely no” energy and 100 is “definitely so”

(1) Mechanism of action

(2) Ritalin Package Insert

Predicted Pharmacokinetics for Two Potential Dosing Regimens of SDX (Once Daily or B.I.D)



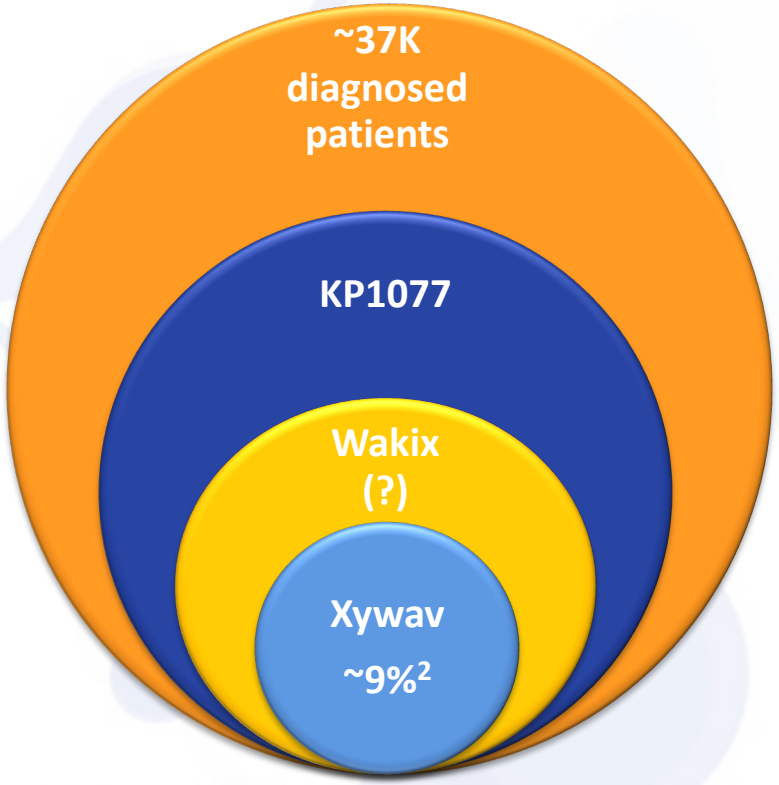
Plasma concentrations were estimated based on data collected in study KP879.101

Predicted PK is shown for steady state of 240 mg SDX based on single oral dose of 240 mg SDX Cl 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/

Business Development Focus

Pipeline Expansion through Internal and External Opportunities

Leveraging Internal Candidates for Multiple Indications

Arimoclomol has shown potential effectiveness in other indications related to lysosomal storage disorders such as Gaucher Disease, which is another rare indication that severely affects the liver and spleen.

Serdemethylphenidate (SDX) provides an opportunity to explore indications outside ADHD and IH:

- SDX is the only C-IV methylphenidate-based product; all others are C-II
- SDX has a unique PK profile allowing for gradual and continuous release throughout the day
- Currently, there is no generic equivalent and not substitutable

- SDX should provide benefit to patients with both Type I and II narcolepsy
  - Initiate clinical trial shortly after IH trial initiation

- Recent trial data suggested SDX could be a treatment option for Stimulant Use Disorder (SUD)
  - KP879 clinical trial data was compelling, development will be challenging and lengthy
  - Seeking partnership with government, academia and/or industry to advance

Pipeline Expansion Strategy to Accelerate Value Creation

- Our strategic focus (internal and external) is guided by these criteria:
  - **Commercial Opportunity** (physician/KOL input, payor research, competitive landscape)
  - **Risk** (clinical, development, regulatory)
  - **Time, Cost and Need** (cost of development, timeline to approval, strategic considerations)

- External focus is primarily within the broad CNS/rare diseases spaces, for example:
  - **Neurology and neurodegenerative diseases**: Alzheimer's, Parkinson's and Huntington's Disease
  - **Psychiatric disorders**: indications focused on more niche market opportunities like psychedelics
  - **Rare diseases** and other niche markets
  - **Adjacent or related therapeutic categories**: gastroenterology, metabolic diseases, endocrinology

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- Seeking assets in Phase 2 stage or later for in-licensing/acquisition:
  - Later-stage clinical candidates add catalysts, drive investor interest and potential for value creation
  - Multi-channel development program diversifies risks and adds multiple products for potential diversified commercialization strategy

AZSTARYS®

d-Methylphenidate Prodrug Product for the Treatment of ADHD

AZSTARYS® Product Highlights

- 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
  - **SDX is a Schedule IV compound; the first-and-only C-IV methylphenidate-based compound**
  - 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

IMPORTANT SAFETY INFORMATION, Contraindications, Warnings and Precautions, Adverse Reactions and Drug Interactions may be found within the full Prescribing Information at www.kempharm.com/pipeline-products/#kp415

AZSTARYS® - U.S. Commercial Launch

- **July 2021, Corium, an affiliate of GPC, launched AZSTARYS (serdexmethylphenidate and dexmethylphenidate capsules) in the U.S.**
  - **As of Mar 1, 2021, over 110 million commercial and Medicaid** lives have access to AZSTARYS[1]
  - Recent wins in payor access have contributed to Corium accelerating its national rollout of AZSTARYS
  - Corium continues to grow its presence in the market, expanding its team to support the national product launch

- **AZSTARYS Commercial Launch is a Significant Opportunity for KemPharm**
  - License agreement with Commave, an affiliate of GPC, provides significant economic benefits to KemPharm tied to the commercialization of AZSTARYS
  - *Accelerating launch efforts will support KemPharm's potential for earning sales milestones in 2022*

Source: (1) Estimate from Corium, Inc.

Financial Update

Q1 2022 Results; Financial Position is a Source of Strength

Income Statement Details for Q1 2022:

- Q1 2022 revenue of $4.0M, derived primarily from consulting services fees, royalties, and a success fee from Corium related to FDA approval of its product, ADLARITY
- Q1 2022 net loss attributable to common stockholders of ($1.9M), or ($0.05) per basic and diluted share
- Looking ahead, we expect R&D expense will continue to increase through FY 2022 as the KP1077 development program continues forward, and revenue will also be added beginning in Q2 2022 from the arimoclomol French EAP

Balance Sheet Details as of March 31, 2022:

- Cash, cash equivalents, marketable securities and long-term investments was $119.1M
- Line of credit for the arimoclomol acquisition expected to be serviced by French EAP cashflow
- Available cash, cash equivalents, marketable securities and long-term investments extends cash runway beyond 2025

Upcoming Clinical, Reg and BD Milestones Create Potential Near-Term Value

Milestone	Q1 2022	Q2 2022	Q3 2022	Q4 2022	Q1 2023	Q2 2023
Arimoclomol						
Re-file NDA for NPC					x	
KP1077 for IH						
Type B meeting with FDA	✓					
IND filing/may proceed		✓				
Phase 1 CV differentiation trial		✓	x			
Phase 2 trial			x			x
KP1077 for Narcolepsy						
Type B meeting with FDA			x			
IND filing				x		
Phase 2/3 trial initiation				x		
KP879						
Final trial results	✓	*Seeking external funding and/or collaborations…*				

*Note: "X" denotes an event, **blue** box denotes activity timeframe*

KemPharm: Looking Ahead

- ✓ NDA-stage, revenue-generating asset expands pipeline targeting rare CNS diseases
- ✓ "Capital efficient" financial structure, potential to create positive cash flow, and no shareholder dilution
- ✓ Potential to re-file NDA as soon as **Q1 2023**

Arimoclomol Acquisition

KP1077 Development Program

- ✓ KP1077 IND for IH submitted to FDA
- ✓ Phase 2 trial initiation in IH in 2H 2022
- ✓ Cardiovascular trial initiated; Data Q3 2022

- ✓ Full national team in place
- ✓ 110M+ covered commercial lives
- ✓ Expanded launch of AZSTARYS supports revenue potential from royalties and milestones in 2022

AZSTARYS® Now Launched Nationally

Strong Balance Sheet to Support Value Creation

- ✓ Cash, cash equivalents and long-term investments of **$119.1M** as of Mar. 31, 2022
- ✓ Solid balance sheet supports development efforts and other pipeline expansion activities
- ✓ Available capital extends cash runway beyond 2025

KemPharm INC

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