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## KEMPHARM VALUE PROPOSITION

Innovative
pharmaceutical
company discovering
and developing novel
treatments for CNS and
rare diseases

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

Focus on high-value areas with significant unmet needs in CNS/rare disease with potential to internally commercialize

### **Experienced Management Team in Corporate and Drug Development**

## 







#### **Collective Team Experience:**

























# KemPharm Leverages its LAT® Prodrug Technology to Improve the Attributes of Approved Drugs



- Select FDA-approved and widely prescribed drug for improvement, seek indications with few options or significant unmet need
- Chemically modify using a ligand to create a prodrug
  - Ligands GRAS or demonstrated to be safe
  - Prodrugs generate composition-based patents
- 3 Following ingestion, normal human metabolic processes cleave the ligand and release the active drug
  - Generates long-lived composition-of-matter patent protection
  - Proprietary to KemPharm

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

Strategic Focus on CNS/Rare Disease	<ul> <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 CNS/rare disease with potential to internally commercialize</li> </ul>
KP1077 for the Treatment of Idiopathic Hypersomnia (IH)	<ul> <li>✓ High-value opportunity with significant unmet need; represents potential for meaningful near-term value</li> <li>✓ Potential KemPharm commercial candidate</li> </ul>
Other SDX Product Opportunities	<ul> <li>✓ Versatility of the SDX family of product candidates could unlock significant value; "pipeline in a pill"</li> <li>✓ Multiple potential indications with initial focus in sleep disorders</li> </ul>
AZSTARYS® License	✓ Expanding launch of AZSTARYS provides ongoing revenue potential from royalties and milestones
Strong Balance Sheet	<ul> <li>✓ Cash and equivalents of \$127.8M as of Dec 31, 2021</li> <li>✓ Strong cash position supports development plan and other opportunities</li> <li>✓ Combined with forecasted revenues, cash runway extends to 2025 and beyond</li> </ul>

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

Indication	Product Candidate	Phase of Development	Anticipated Timing of Next Milestone				
Rare Sleep Disorders							
Idiopathic Hypersomnia (IH)	KP1077 Phase 2 Q3 20		Q3 2022				
Narcolepsy Type I and II	KP1077	Phase 2	Q4 2022				
Sleep Disorders	TBD	In-licensing, acquisition or internal candidate	H2 2022				
First-in-Class Therapy							
Stimulant Use Disorder (SUD)	KP879	Final Phase 1 Data	Q1 2022				
In-licensed or Acquired Product(s)							
CNS or Related	TBD	Phase 2 or later H2 2022					



## **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<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 are highly debilitating <u>IH can be more debilitating than narcolepsy</u>
  - 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
  - o 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<sup>(3)</sup>

Sources: (1) https://doi.org/10.1093/sleep/zsy061.624

(3) https://www.sleepcountshcp.com/idiopathic-hypersomnia-treatment-options

(2) <a href="https://www.sleepcountshcp.com/what-is-idiopathic-hypersomnia">https://www.sleepcountshcp.com/what-is-idiopathic-hypersomnia</a>

#### **KP1077 Product Candidate Overview**

- 100% Serdexmethylphenidate (SDX) product with multiple dosing options depending on patient needs
  - Dosed either QD (once at bedtime) or BID (twice 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
- Orphan drug designation potential
  - Fast-track eligible
  - Break-through designation eligible
- 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	<ul> <li>Approved for IH; centrally acting depressant</li> <li>Dosed twice at night; once before bed and another 4 hrs later</li> <li>75% of patients in Xywav IH trial maintained or added stimulant treatment</li> </ul>	Highest dose: \$187,000/year
Provigil/Nuvigil® (modafinil/armodafinil)*	Teva	C-IV	<ul> <li>Approved for treatment of EDS associated with narcolepsy</li> <li>Numerous drug-drug interactions including with hormonal contraception and antidepressants</li> <li>Serious adverse events include Stevens-Johnson Syndrome, angioedema, anaphylaxis and multi-organ hypersensitivity</li> </ul>	Provigil: \$24,000/year
Various IR/ER methylphenidate products*	Various brands and generics	C-II	<ul> <li>Ritalin<sup>®</sup> indicated for the treatment of narcolepsy</li> <li>Ritalin daily dose not to exceed 60 mg</li> <li>Elevated blood pressure and heart rate; serious cardiovascular effects may also occur</li> </ul>	Varies: ~\$4,000-\$5,000/year
Wakix (pitolisant)*	Harmony Biosciences	Not Scheduled	<ul> <li>Approved for treatment of EDS or cataplexy in narcolepsy</li> <li>Significant drug-drug interactions including antidepressants and antihistamines</li> <li>Contraindicated in severe hepatic impairment</li> <li>QT interval prolongation</li> </ul>	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
- o "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<sub>max</sub>) 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)
- o Only one other product, Wakix® (pitolisant), is under development in IH

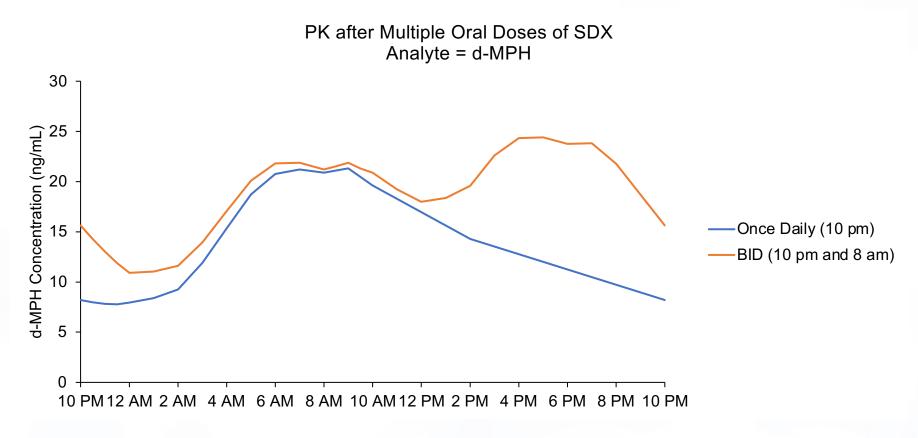


### **KP1077 Value Proposition: Addressing Key Unmet Needs**

- Many comorbidities and patient demographics complicate treatment; current off-label treatment options have significant limitations and provide limited symptom relief
  - o Brain fog 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
  - Modafinil/armodafinil can interfere with contraception:
    - SDX does not have drug interactions with contraception
  - Depression is a common comorbidity with IH; modafinil/armodafinil and Wakix<sup>®</sup> both have significant drug interactions with the most commonly prescribed antidepressants:
    - SDX does not interfere with antidepressant metabolism

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

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



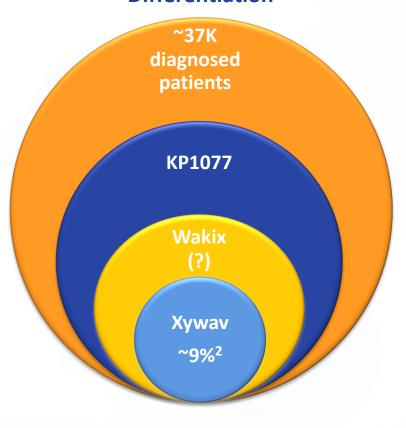
Plasma concentrations were estimated based on data collected in study KP879.101

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

<sup>(2) &</sup>lt;a href="https://investor.jazzpharma.com/investors/events-presentations">https://investor.jazzpharma.com/investors/events-presentations</a>

<sup>(3) &</sup>lt;a href="https://www.reuters.com/business/healthcare-pharmaceuticals/us-fda-approves-jazz-pharmas-drug-excessive-daytime-sleepiness-2021-08-12/">https://www.reuters.com/business/healthcare-pharmaceuticals/us-fda-approves-jazz-pharmas-drug-excessive-daytime-sleepiness-2021-08-12/</a>

## **Business Development Focus**

Maximizing Serdexmethylphenidate Value Potential Pipeline Additions Through In-Licensing



### Serdexmethylphenidate (SDX) Platform Potential

- SDX also provides an opportunity to explore indications outside ADHD and IH
  - o 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 potentially be a treatment option for Stimulant Use Disorder (SUD)
  - KP879 clinical trial data was compelling and scientific rationale still exists
  - Challenging and lengthy development program will be required
  - Seeking partnership with government, academia and/or industry to advance



### **Pipeline Expansion Strategy to Accelerate Value Creation**

- Our strategic focus, including review of internal development candidates, 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 space, including these examples:
  - 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
- Seeking assets in Phase 2 stage or later, subject to our evaluation criteria, for in-licensing/acquisition
  - Later stage clinical candidates can add clinical trial data catalysts, driving investor interest and, if successful, potential for value creation
  - Multi-channel development program with multiple product candidates diversifies risks and adds products for potential commercialization

## **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 <a href="https://www.kempharm.com/pipeline-products/#kp415">www.kempharm.com/pipeline-products/#kp415</a>

### **AZSTARYS® - U.S. Commercial Launch**

- July 2021, Corium, an affiliate of GPC, launched AZSTARYS (serdexmethylphenidate and dexmethylphenidate capsules) in the U.S.
  - As of Jan 1, 2021, over 100 million commercial and Medicaid lives have access to AZSTARYS, which is 2x coverage from Oct 1, 2021<sup>1</sup>
  - Recent wins in payor access have contributed to Corium accelerating its national rollout of AZSTARYS
  - Full national field team staffing expected to be in place by end of Jan 2022 to support national rollout planned for Q1 2022
- 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



### **License Agreement with Commave (Affiliate of GPC)**

## License agreement with Commave, an affiliate of GPC, was entered into Sept 2019 for AZSTARYS® and KP484

- Commercial rights assigned to Corium, another affiliate of GPC, led by ex-Shire team
  - Perry Sternberg (CEO) and key commercial team members led Vyvanse commercial effort at Shire
- Total potential regulatory and sales milestone payments including payments already made: \$590M
  - \$35M in regulatory milestones already paid
  - Sales milestones to be paid based on tiers
- Royalty rates range from a percentage in the high single digits up to the mid-twenties for U.S. net sales
- ROFR and ROFN for SDX-based products
- ROFN for amphetamine-based prodrug products

# **Financial Update**



### Q3 2021 Results; Balance Sheet Demonstrates Solid Financial Position

- Revenue of \$2.0M, derived primarily from consulting service fees
- Net loss (\$1.8M), or (\$0.05) per basic and diluted share
- Net operating loss was (\$2.2M), which increased by \$1.0M compared to Q3 2020 due to an increase in operating expenses
  - R&D expenses were \$2.2M, an increase of \$0.5M compared to Q3 2020 due to the ongoing SDX study
  - G&A expenses were \$1.9M, an increase of \$0.5M compared to Q3 2020
- Balance sheet details as of Dec. 31, 2021:
  - Cash and cash equivalents was \$127.8M as of December 31, 2021
  - Available capital combined with revenues extends cash runway through 2025 and beyond
  - \$50M share repurchase program in place through 2023
  - o ATM has not been utilized and is available for targeted uses, but only if needed
  - o We intend to convert current S-1 to an S-3 to eliminate requirement for continuous updated filing



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

Milestone	Q1 2022	Q2 2022	Q3 2022	Q4 2022	Q1 2023	Q2 2023
KP1077 for IH						
Type B meeting with FDA	Х					
IND filing			х			
Phase 1 CV differentiation trial		х	х			
Phase 2 trial			х			х
KP1077 for Narcolepsy						
Type B meeting with FDA			х			
IND filing				х		
Phase 2/3 trial initiation				х		
KP879						
Final trial results	х					
Additional clinical stage candidate(s)						

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

### **KemPharm: Looking Ahead**

- ✓ KP1077: Substantial high-value opportunity with significant unmet need
- ✓ IND filing in mid-2022, Phase 2 trial initiation as early as Q3 2022

Initiation of KP1077
Development Program

Opportunities to Further Expand Pipeline

- ✓ Continuing efforts to build a highly differentiated pipeline of development assets
- ✓ Focused on high-value areas with significant unmet needs in CNS/rare disease with potential to internally commercialize

- ✓ Full national team in place by end of Jan 2022
- √ 100M+ covered commercial and Medicaid lives as of Dec 31, 2021
- ✓ Expanding launch of AZSTARYS supports revenue potential from royalties and milestones in 2022

AZSTARYS®
Launch
Gaining
Traction

Strong
Balance Sheet
to Support
Value Creation

- ✓ Cash and cash equivalents of \$127.8M as of Dec 31, 2021
- ✓ Solid balance sheet supports development efforts and other pipeline expansion activities
- ✓ Available capital + revenues extends cash runway through 2025 and beyond

