

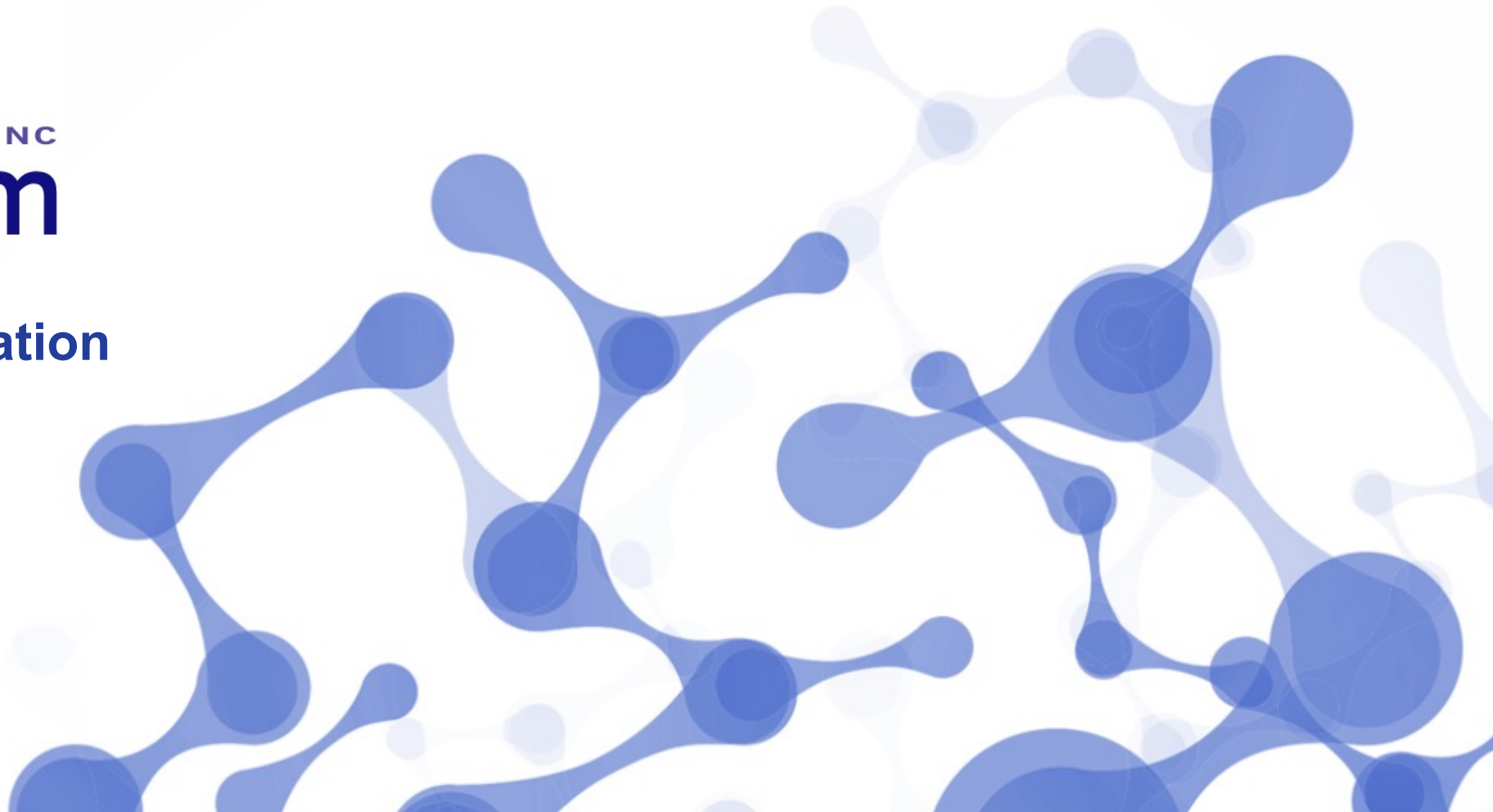


INC

**KemPharm**

**Management Presentation**

**September 2021**



## Cautionary Note Regarding Presentation Information

This presentation may contain forward-looking statements made in reliance upon the safe harbor provisions of Section 27A of the Securities Act of 1933, as amended, and Section 21E of the Securities Exchange Act of 1934, as amended. 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” or the negative versions of those words or other comparable words. Forward-looking statements are not guarantees of future actions or performance. These forward-looking statements include statements regarding the timing of the potential commercial launch of AZSTARYS, the market outlook for AZSTARYS, potential regulatory and sales milestone and royalty payments pursuant to the License Agreement with an affiliate of Gurnet Point Capital, the potential benefits of AZSTARYS, the clinical development of KP879, the potential benefits of SDX being classified as a Schedule IV controlled substance, and KemPharm’s forecasted cash runway. 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 uncertainties and risks that could significantly affect current plans. Risks concerning KemPharm’s business are described in detail in the “Risk Factors” section of KemPharm’s Annual Report on Form 10-K for the year ended December 31, 2020, KemPharm’s Quarterly Report for the quarter ended June 30, 2021, and KemPharm’s other filings with the Securities and Exchange Commission. KemPharm is under no obligation to, and expressly disclaims any such obligation to, update or alter its forward-looking statements, whether as a result of new information, future events or otherwise.

This presentation also may contain estimates and other statistical data made by independent parties and by us relating to market size and other data about our industry. This data involves a number of assumptions and limitations, and you are cautioned not to give undue weight to such estimates. In addition, projections, assumptions and estimates of our future performance and the future performance of the markets in which we operate are necessarily subject to a high degree of uncertainty and risk.



# KEMPHARM VALUE PROPOSITION

Specialty pharmaceutical company discovering and developing novel treatments for CNS diseases

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

Leveraging LAT® Platform Technology and extensive CNS expertise to create a pipeline of high-value opportunities

# Experienced Management Team in Corporate and Drug Development

Travis C. Mickle, PhD  
President and CEO



R. LaDuane Clifton  
CFO



Sven Guenther, PhD  
EVP of R&D



## Collective Team Experience



# KemPharm Leverages its LAT<sup>®</sup> Prodrug Technology to Improve the Attributes of Approved Drugs



- 1 Select FDA-approved and widely prescribed drug for improvement, seek indications with few options or significant unmet need
- 2 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

## Pipeline of Multiple Product and Product Candidates

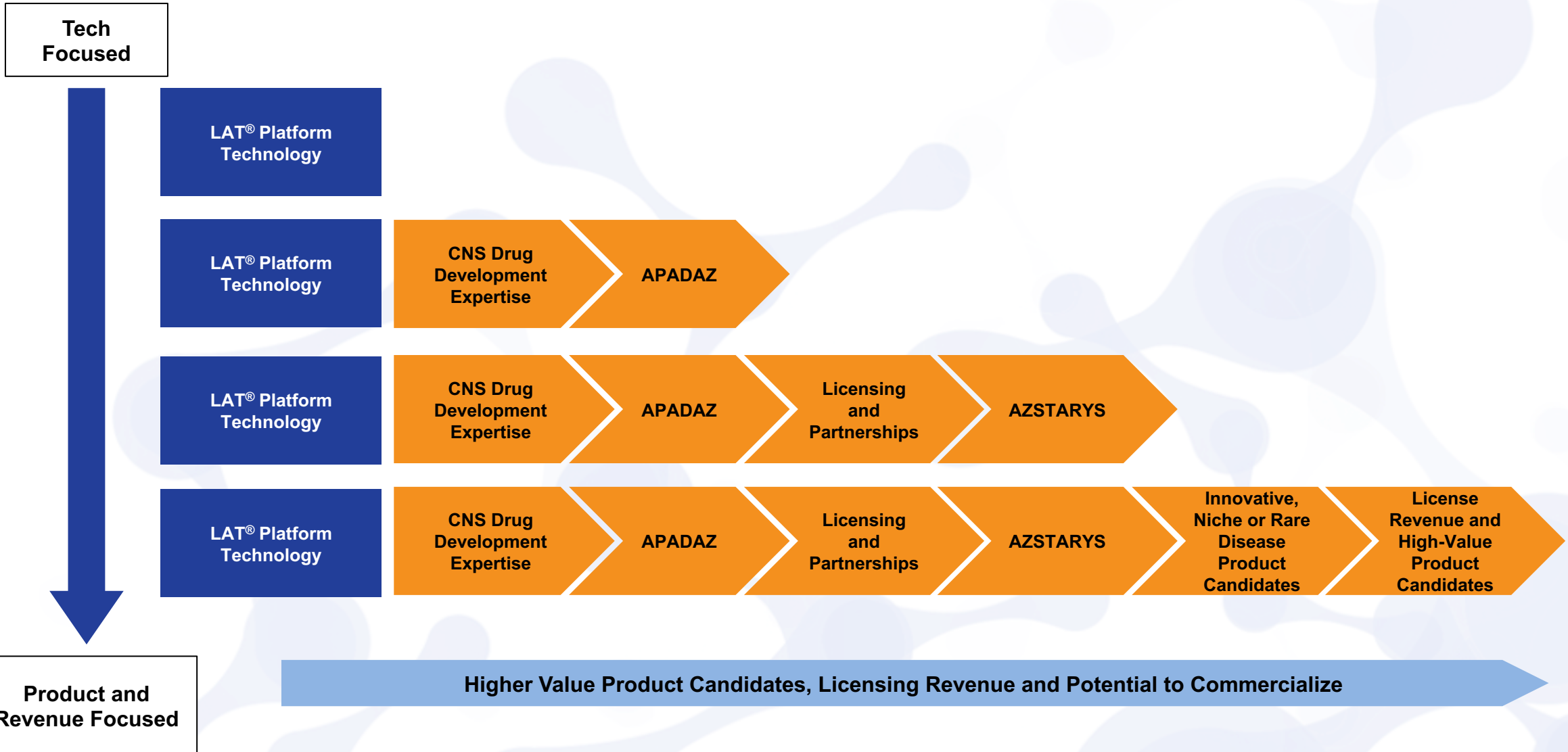
Indication	Product Candidate	Parent Drug	Next Milestone	Potential Timing of Next Milestone	Potential NDA Submission
ADHD	AZSTARYS™	Methylphenidate (ER)	Royalties and Sales Milestones	2022	NA
ADHD	KP484	Methylphenidate (ER)	Initiation of Efficacy Trial	TBD by Partner	As early as 2023
<b>First-in-Class Therapy</b>					
Stimulant Use Disorder (SUD)	KP879	Methylphenidate (ER)	Phase 1 Data	YE 2021	2024
<b>Rare CNS Diseases</b>					
Idiopathic Hypersomnia (IH)	KP1077	Methylphenidate (ER)	Phase 2/3	1H 2022	2025

*Multiple preclinical candidates under evaluation for addition to pipeline*

## Partnered Assets Create the Potential For Near and Long-Term Value

Candidate	Indication	Partner	Status
<b>AZSTARYS™</b> Methylphenidate ER	ADHD	Gurnet Point Capital/Corium	<ul style="list-style-type: none"> <li>• <b>Commercially available</b></li> <li>• Royalties to follow</li> </ul>
<b>KP484</b> Methylphenidate ER	ADHD	Gurnet Point Capital/Corium	<ul style="list-style-type: none"> <li>• <b>Licensed</b></li> <li>• Ready to enter clinical phase</li> <li>• Timing TBD with partner</li> </ul>
<b>SDX-Candidates</b> Methylphenidate	Various (SUD, IH)	Gurnet Point Capital/Corium	<ul style="list-style-type: none"> <li>• IND cleared for KP879</li> <li>• Pre-clinical not needed, clinical underway 2021</li> <li>• GPC has Right of First Negotiation after POC, then one-time ROFR up to NDA acceptance</li> </ul>
<b>KP922</b> Amphetamine	TBD	Gurnet Point Capital/Corium	<ul style="list-style-type: none"> <li>• Timing TBD</li> <li>• GPC has Right of First Negotiation after POC, but no ROFR</li> </ul>
<b>APADAZ®</b> Hydrocodone IR	Pain	KVK Tech	<ul style="list-style-type: none"> <li>• <b>Licensed</b></li> <li>• Commercial launch Q4 2020</li> <li>• Potential for \$3.4M in m/s and reimb, plus profit share up to 50%</li> </ul>

# Evolution of the KemPharm Strategy Creates Risk Mitigated Value





# KemPharm: Recent Highlights

- ✓ Commercial launch on July 21, 2021
- ✓ Expect royalties and sales milestones in 2022 and beyond
- ✓ Tiered royalty rates on U.S. net sales of high single digits up to mid-twenties
- ✓ AZSTARYS™ patents extend to 2037

AZSTARYS™

Solid  
Financial  
Position

- ✓ Cash on hand as of Jun 30, 2021 = \$132.3M
- ✓ Q2 2021 net income of \$6.2M, or \$0.18 per basic share
- ✓ Combination of AZSTARYS-related regulatory milestones and June warrant transaction bolstered cash reserves

- ✓ SDX classified as Schedule IV Controlled Substance by the DEA
- ✓ HHS and DEA determined that SDX has generally low potential for abuse and a lower potential for abuse compared to d-MPH
- ✓ Key differentiator for AZSTARYS<sup>1</sup>, and all other SDX-based product candidates

SDX  
Schedule IV

Beyond  
AZSTARYS

- ✓ Initiated clinical trial with SDX, with data expected prior to year-end 2021
- ✓ KVK-Tech preparing to expand Sure Med collaboration for APADAZ®, Perspectives in Care™, into additional regions

<sup>1</sup> AZSTARYS is a Schedule II controlled substance which contains SDX, a Schedule IV prodrug of d-methylphenidate

**AZSTARYS™**

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



## ADHD Market

- ~\$17.5 billion ADHD market with prescription growth of >4% year-over-year
- The branded portion of the ADHD market was ~\$7.4B in 2019 and more than 95% of these branded prescriptions are for extended release
- Methylphenidate (MPH) accounted for approximately 20 million TRx's and \$4.9 billion in sales in 2019
- Early market research indicates prescribers see the following product benefits as key advantages:
  - Duration of action (60%)
  - Lower abuse potential (52%)
  - Early onset of action (43%)
- Market research also indicates that prescribers estimate that MPH is given as the preferred first line of therapy for children under the age of 13 approximately 60% of the time



# 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](http://www.kempharm.com/pipeline-products/#kp415)**



## Selected Extended-Release Stimulant Products for ADHD<sup>(1)</sup>

Brand Name Active Ingredient	Sponsor	Dosage Form	Dosage Strengths	Features	Reduced Drug Liking
Vyvanse® (lisdexamfetamine)	Shire	Capsule & Chewable	10, 20, 30, 40, 50, 60 & 70 (cap only) mg	<ul style="list-style-type: none"> <li>• Prodrug of d-amphetamine</li> <li>• Ages: 6 years and older</li> <li>• Onset: <b>1.5 hours</b></li> <li>• Duration: 13 hours</li> <li>• Indicated for binge eating disorder</li> </ul>	<b>Some Difference</b>
Adderall XR® (mixed salts of AMPH)	Shire	ER Capsule	5, 10, 15, 20, 25 & 30 mg	<ul style="list-style-type: none"> <li>• Comprised of both L-AMPH &amp; D-AMPH</li> <li>• Ages: 6 years and older</li> <li>• Extended-release profile</li> <li>• <b>No duration indicated</b></li> </ul>	<b>No</b>
Focalin XR® (dexamethylphenidate)	Novartis	ER Capsule	5, 10, 15, 20, 25, 30, 35 & 40 mg	<ul style="list-style-type: none"> <li>• Ages: 6 years and older</li> <li>• Onset: 0.5 hours</li> <li>• Duration: <b>12 hours</b></li> </ul>	<b>No</b>
Concerta® (methylphenidate)	Janssen	ER Tablet	18, 27, 36 & 54 mg	<ul style="list-style-type: none"> <li>• Ages: 6 years to 65 years</li> <li>• Onset: <b>2 hours</b></li> <li>• Duration: <b>12 hours</b></li> </ul>	<b>Small Difference</b>

(1) Information located within each respective package insert

# AZSTARYS™ Label

## Sections 1 and 2.3

### Section 1: Indications and Usage

- AZSTARYS is indicated for the treatment of Attention Deficit Hyperactivity Disorder (ADHD) in patients 6 years of age and older.

### Section 2.3: Administration Information

- Administer AZSTARYS orally once daily in the morning with or without food.
- AZSTARYS capsules may be taken whole, or opened and the entire contents sprinkled into 50 mL of water or over 2 tablespoons of applesauce.

**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](http://www.kempharm.com/pipeline-products/#kp415)**



## Section 6

### Section 6.1: Clinical Trials Experience

To adjust for normal growth, z-scores were derived (measured in standard deviations [SD]); z-scores normalize for the natural growth of children and adolescents by comparisons to age- and sex-matched population standards. A z-score change less than 0.5 SD is considered not clinically significant.

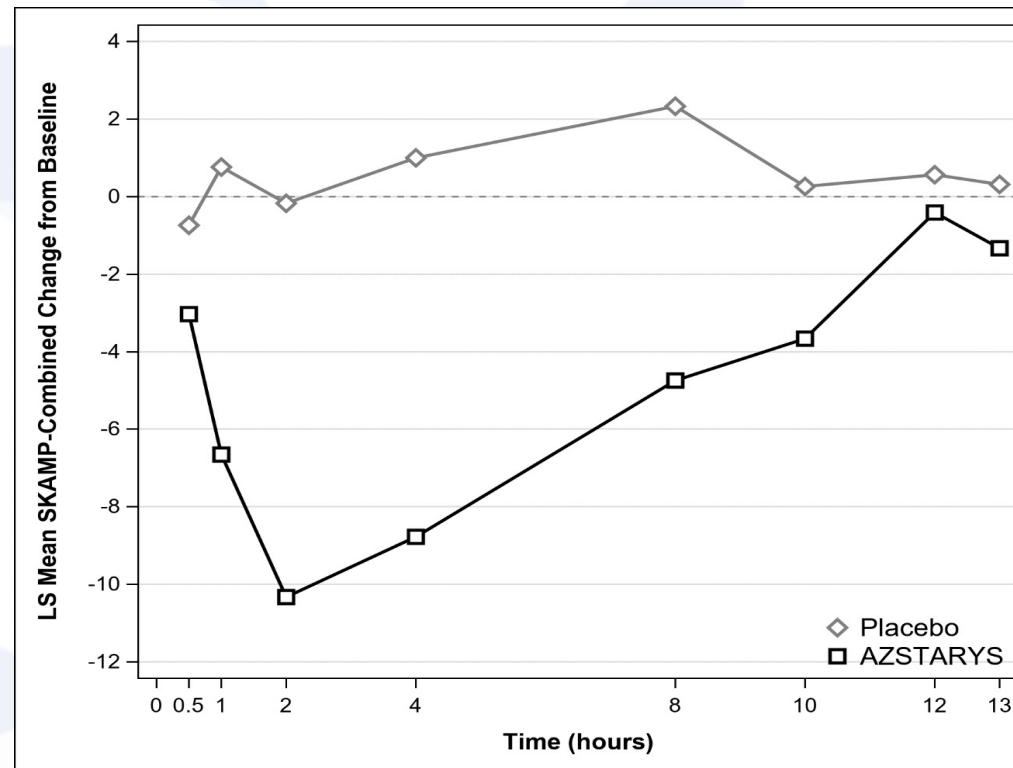
In this study, the mean increase in weight from baseline to Month 12 was 3.4 kg among study completers. The mean change in z-score from baseline to Month 12 was -0.20, indicating a lower than expected increase in body weight compared to children of the same age and sex, on average. Most of the weight z-score decline occurred in the first 4 months of treatment.

The mean increase in height from baseline to Month 12 was 4.9 cm among completers. Using the same z-score analysis for height, the mean change in z-score from baseline to Month 12 was -0.21, indicating a lower than expected increase in height compared to pediatric patients of the same age and sex, on average.

**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](http://www.kempharm.com/pipeline-products/#kp415)**

## Section 14

**Figure 2: LS Mean Change in SKAMP-Combined Score from Baseline after Treatment with AZSTARYS or Placebo during Classroom Day in Pediatric Patients (6 to 12 years) with ADHD**



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# ASTARYS™ - U.S. Commercial Launch

- **July 2021, Corium, an affiliate of GPC, launched AZSTARYS™ (serdexmethylphenidate and dexmethylphenidate capsules) in the U.S.**
  - Launch initially focused on a few states with select prescribers due to typical NDC blocks with most payors
  - Payor discussions ongoing; typical timeline starts to see progress roughly 6-months post-launch
  - Initial positive signs
    - No push-back from prescribers or patients regarding the clinical profile
    - Repeat prescribing and refills
  - Initial launch may have been slowed by COVID-19 outbreak in key states (TX, FL)
- **AZSTARYS Commercial Launch is a Significant Milestone for KemPharm**
  - Further demonstrates value potential of SDX and KemPharm's LAT® platform
  - License agreement with Commave, an affiliate of GPC, provides significant economic benefits to KemPharm tied to the commercialization of AZSTARYS
  - Based on the approved label for AZSTARYS, we believe peak market share may be greater than original internal forecast



## 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



# **Serdexmethylphenidate (SDX)**

**First-and-Only C-IV Methylphenidate-Based Stimulant**



## Serdexmethylphenidate (SDX) – Schedule IV Classification

- SDX classified as a Schedule IV Controlled Substance by DEA
  - AZSTARYS™ classified as a Schedule II controlled substance as it includes a 70:30 mixture of SDX (Schedule IV) and d-MPH (Schedule II), respectively
- SDX Schedule IV classification based on eight-factor analysis by HHS, which concluded that, “SDX is related in action and effect to the schedule IV substance phentermine, and can therefore be expected to have a similar potential for abuse.”
- HHS also affirmed that, “in clinical studies, SDX demonstrated a lower potential for abuse when compared to d-MPH.”
- SDX is the sole API in two other potential product candidates, KP879 and KP1077, that are currently under evaluation



## Serdexmethylphenidate (SDX) Opportunity

- SDX provides an opportunity to explore indications outside ADHD
  - 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
  - No generic equivalent and not substitutable
- Recently initiated a trial with SDX under the KP879 IND exploring the pharmacokinetics, safety and exploratory effects of SDX at doses above those studied with AZSTARYS™ (>240 mg)
  - Data release should be available prior to year-end 2021
- Goal is to assess the best potential clinical path with SDX in future drug development in such indications like Stimulant Use Disorder (SUD), Idiopathic Hypersomnia (IH) and others
- Provide additional clarification as to the potential development of SDX in the near-future once clinical results are available
  - Science and data will drive further investigation
  - May need to prioritize based on potential commercial value, timing, cost and overall clinical risk



# **SDX Potential Product Candidate: KP879**

**Agonist Replacement Therapy for the Treatment of Stimulant Use Disorder (SUD)**



## Stimulant Use Disorder (SUD)

- Stimulant Use Disorder (SUD) is broadly defined as the abuse or misuse of cocaine, methamphetamine, or other stimulants
- Although there are therapies for opioid addiction (buprenorphine, methadone), there are currently no approved treatments for SUD
- Studies with agonist replacement therapies have shown promising data for treating SUD

### U.S. Prevalence of Abuse in 2018

Stimulant	Abuse/Misuse Reported In Last 30 Days
<b>Total Stimulant Abuse/Misuse</b>	<b>4,600,000</b>
Cocaine <sup>a</sup>	1,900,000
Methamphetamine <sup>b</sup>	1,000,000
Rx Stimulants	1,700,000
<b>Rx Pain Relievers (incl. opioids)</b>	<b>2,900,000</b>

<sup>a</sup> includes crack cocaine; <sup>b</sup> includes only illicitly manufactured methamphetamine

Source: Substance Abuse and Mental Health Services Administration. (2019). HHS Publication No. PEP19-5068, NSDUH Series H-54.

## KP879 for the Treatment of SUD

- Potential KP879 features and benefits:
  - Stand-alone formulation of serdexmethylphenidate (SDX)
  - Releases d-methylphenidate (d-MPH), a dopamine reuptake inhibitor (similar pharmacology as abused stimulants)
  - Very gradual onset of blood concentrations of released d-MPH followed by sustained release
  - Low oral, IN, and IV abuse potential
- Focus of initial clinical studies
  - High dose PK
  - High dose safety
  - Effect size in different treatment populations
- May qualify for FDA fast track, breakthrough therapy and/or priority review





# **SDX Potential Product Candidate: KP1077**

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



## Idiopathic Hypersomnia (IH)

- Rare disease with an estimated 37,000 US patients
- There are no approved therapies for the treatment of Excessive Daytime Sleepiness (EDS) in IH
- Narcolepsy treatments have commonly been used for treating IH symptoms
  - GHB/sodium oxybate for improved sleep; recent approval of XYWAV®
  - Stimulants for Excessive Daytime Sleepiness (EDS) are used off-label
    - Methylphenidate remains the most commonly used stimulant for IH
    - Amphetamine
    - Modafinil
- Unmet needs are focused on symptom control and safety
  - No 16-hour product available for EDS symptom control
  - PRN (as needed) utilization of IR methylphenidate is very typical for “break-through” sleepiness
  - Potential for abuse remains a concern of most prescribers



# KP1077 Product Candidate Overview

- Once-daily dosing
- Potential features and benefits
  - Single dose allows for symptom control up to 16 hours
  - Still allows for PRN stimulant use for “break-through” sleepiness
  - C-IV designation
- Orphan drug designation potential
  - Fast-track eligible
  - Break-through designation eligible
- No generic equivalent; not substitutable
- Composition-of-matter based patents expire in 2037; additional applications may extend past that time



# Financial Update

As of June 30, 2021



## Q2 2021 Financial Results

- Revenue of \$12.0M, comprised of \$10.0M milestone payment for DEA scheduling of SDX, and service fee revenue of \$2.0M
- Net income of \$6.2M, or \$0.18 per basic share, compared to net income of \$0.9M, or \$0.21 per basic share and diluted share for Q2 2020
  - Operating income of \$5.8M and non-cash gain of \$0.8M from the forgiveness of the PPP loan during Q2 2021
  - Net loss attributable to common stockholders for Q2 2021 was (\$0.40) per basic and diluted share, driven by a non-cash deemed dividend of \$16.9M related the warrant exercise inducement transaction in June
- Operating income increased \$3.2M, primarily driven by an increase in revenue of \$5.1M, partially offset by a net increase in operating expenses of \$1.8M as compared to Q2 2020
  - R&D expenses were \$2.8M, an increase of \$0.9M vs. Q2 2020
  - G&A expenses were \$2.3M, an increase of \$0.6M compared to Q2 2020



## Q2 2021 Balance Sheet Demonstrates a Solid Financial Position

- As of June 30, 2021, total cash was \$132.3M, an increase of \$56.3M compared to Mar 31, 2021, primarily due to receipt of \$20M in cash milestones related to AZSTARYS, and \$40.6M in proceeds from warrant exercises during Q2 2021
- Completed warrant exercise inducement transaction in June 2021:
  - Received \$39.1M of gross proceeds from the exercise of 6,117,509 existing warrants at exercise price of \$6.36 per share
  - Issued 1,529,379 inducement warrants with an exercise price of \$16.50
- As of June 30, 2021:
  - 34,977,923 common shares outstanding
- KemPharm's balance sheet has been completely restructured and recapitalized with a cash balance that provides for:
  - operating requirements,
  - internal development opportunities, and
  - other potential external investments (in-licensing, acquisition, partnerships, etc.)



# KemPharm: Looking Ahead

- ✓ U.S. commercial rollout is being led by Corium and is progressing as planned
- ✓ KemPharm continues to support manufacturing, scientific and regulatory affairs
- ✓ Expect royalties and sales milestones in 2022 and beyond

AZSTARYS™

Solid  
Financial  
Position

- ✓ Cash on hand as of June 30, 2021 = \$132.3M
- ✓ Tools in place to enable operating and strategic flexibility
- ✓ Potential of licensing revenue from AZSTARYS royalties and sales milestones in 2022

- ✓ Clinical trial with SDX underway
- ✓ With clinical data, multiple potential paths forward will be fully assessed based on commercial potential and development pathway

SDX  
Opportunity

Beyond  
AZSTARYS

- ✓ Continued evaluation of pipeline and external opportunities
- ✓ Upcoming clinical data from SDX clinical trials and initial SDX development plan

<sup>1</sup> AZSTARYS is a Schedule II controlled substance which contains SDX, a Schedule IV prodrug of d-methylphenidate



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

**Leveraging our LAT<sup>®</sup> Prodrug Technology  
to Create Long-Term Value**

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