KemPharm

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Sponsored by H.C. Wainwright & Co.

September 2019

Cautionary Note Regarding Presentation Information

This presentation contains forward-looking statements, including statements about any royalty or milestone payments under our license agreement, the exchange of any future principal under our exchange agreement, our plans to develop and commercialize our product candidates, our planned clinical trials for our prodrug product candidates, the timing of and our ability to obtain and maintain regulatory approvals for our product candidates, including expectations about our ability to use the 505(b)(2) pathway and expedited FDA review, the clinical utility of our product candidates, the timing of the APADAZ® launch, the timing of the NDA filing for and the potential approval of KP415 and other product candidates, the plans and capabilities of our collaborators, potential addressable markets for our product candidates, and our intellectual property position. These statements involve substantial known and unknown risks, uncertainties and other factors that may cause our actual results, levels of activity, performance or achievements to be materially different from the information expressed or implied by these forward-looking statements. We may not actually achieve the plans, intentions or expectations disclosed in our forward-looking statements, and you should not place undue reliance on our forward-looking statements. Actual results or events could differ materially from the plans, intentions and expectations disclosed in the forward-looking statements we make. The forward-looking statements in this presentation represent our views as of the date of this presentation. These and other risks concerning our business are described in additional detail in our Quarterly Report on Form 10-Q filed with the SEC on August 13, 2019, and our other Periodic and Current Reports filed with the SEC. We anticipate that subsequent events and developments will cause our views to change. However, while we may elect to update these forward-looking statements at some point in the future, we have no current intention of doing so except to the extent required by applicable law. You should, therefore, not rely on these forward-looking statements as representing our views as of any date subsequent to the date of this presentation. Further, the information contained in this presentation speaks only as the date hereof. While we may elect to update the information in this presentation in the future, we disclaim any obligation to do so except to the extent required by applicable law.

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KMPH: Current Price and Trading Statistics[1]

Ticker	Nasdaq: KMPH
Closing Price (9/6/19)	$0.83
52 Week High / Low	$5.43 / $0.71
Market Capitalization	$25.9M
Common Shares Outstanding	31.3M
50-Day Average Trading Volume	218,798
Fiscal Year-End	December 31
Website	www.kempharm.com

(1) Source: NASDAQ

KemPharm Update: Transformative License Agreement and Improved Financial Position

KP415/KP484 License Agreement - License agreement announced for KP415 and KP484 with up to $493M in upfront and milestone payments plus additional royalties - Up to $73M in upfront and regulatory milestone payments, both prior to and upon approval, based upon timing and final label for KP415, and approval of KP484 - Also includes options for KP879 and KP922	**Debt Restructuring** - Announced debt exchange agreement with Deerfield Management - If all optional debt exchanges are made, total debt of $53.4M remains prior to Feb 2021 maturity, down from $96.3M in May 2018 (45% reduction)
Near-Term NDA, Clinical Data, and Growing New Product Pipeline - KP415 NDA anticipated later this year - KP484 efficacy data in 2020 - KP879 and KP922 represent synergistic new product opportunities	**Increased Cash Runway** - License deal shifts most development costs to partner - If milestones met, license adds capital through KP415 approval and beyond - G&A spending reduced; cost reduction focus continues

KemPharm Leverages its LAT™ Prodrug Technology to Improve the Attributes of Approved Drugs in Large Markets

1) Select FDA-approved and widely prescribed drug for improvement

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 and **applicable across many therapeutic areas**

KemPharm Partnered and Optioned Assets

Indication (Status)	Product Candidate	Parent Drug	Development Status	Next Milestone	Potential Milestone Timing
ADHD (Partnered)	KP415	Methylphenidate (ER)	Clinical	NDA Submission	Q4 2019
	KP484	Methylphenidate (ER)	Clinical	Initiation of Efficacy Trial	2020
SUD* (Optioned)	KP879	Methylphenidate (ER)	Preclinical	IND Submission	Q4 2019
ADHD (Optioned)	KP922	Amphetamine (IR and ER)	Preclinical	IND Submission	2020
Pain (Partnered)	APADAZ®	Hydrocodone/ APAP	FDA Approved	Commercial Launch	Q4 2019

* Stimulant Use Disorder

KP415 and KP484

D-Methylphenidate Prodrug Products for the Treatment of ADHD

KP415/KP484 Collaboration and License Agreement

- ✓ **KemPharm has entered into a definitive collaboration and license agreement with an affiliate of Gurnet Point Capital (GPC)**
- ✓ **Up to a total of $493M in upfront, regulatory, development and sales milestone payments; plus royalty percentages of up to mid-20s of net sales**
  - Licensee granted exclusive worldwide licenses for KP415 and KP484, plus options to add KP879 and product candidates based on KP922, a new prodrug of amphetamine
  - KemPharm to manage all development activities and Licensee responsible for development costs under the license agreement
  - Licensee responsible for commercialization and manufacturing activities, seeking to build a best-in-class CNS sales and managed markets team, with KP415 as its leading product candidate
  - This collaboration is a unique opportunity to bring innovative products to ADHD patients and their families

Summary of Deal Terms

Upfront cash	– $10M, as well as reimbursement of certain pre-approval development costs for KP415
Development costs and activities	– Licensee covers development costs for KP415 post-approval, KP484, and if added, KP879 and KP922 – KemPharm manages development activities
Regulatory milestone payments	– Up to $63M at specified regulatory milestones, both prior to and upon approval, based upon timing and final label for KP415, and approval of KP484
Sales milestone payments	– Payments totaling up to $420M upon achievement of various tiers of annual U.S. Net Sales
Royalty payments	– Tiered royalty payments from a percentage in the high single digits to mid-20s of U.S. Net Sales – Tiered royalty payments from a percentage in the low to mid single digits for Net Sales in each country outside the U.S.

Gurnet Point Capital

- ✓ **GPC is a healthcare fund that invests long-term capital into life sciences and medical technology companies across all stages of project development**
  - Founded by Ernesto Bertarelli, former CEO of Serono, SA, and led by Christopher Viehbacher, Managing Partner and former CEO and board member of Sanofi, who together bring decades of expertise in an industry for which they share a passion
  - GPC brings significant experience at guiding novel drugs through development and commercialization, aided by its unique long-term investment horizon, which helps ensure development and commercial success
  - GPC's investment portfolio includes: Auregen BioTherapeutics, Axcella Health, Before Brands, Boston Pharmaceuticals, Corium International, Innocoll Holdings and Zikani Therapeutics

The ADHD and ER Methylphenidate Market

- ~$12.5 billion ADHD market with prescription growth of >4% year-over-year
- The branded portion of the ADHD market was ~$6.2B in 2018 and more than 95% of these branded prescriptions are for extended release
- Methylphenidate (MPH) accounted for approximately 19.4 million TRx's and $3.9 billion in sales in 2018
- Market research indicates prescribers see the following potential KP415 features 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

Source: Symphony Health, PHAST 2018

KP415 and KP484 Product Overviews

KP415

- Prodrug of d-MPH (SDX) with extended release properties, co-formulated with IR d-MPH
- Potential features and benefits:
  - Once-daily dosing
  - Earlier onset, long duration
  - Lower abuse potential
  - Patient-friendly dosage form
- Potential to be first MPH product approved for pre-school ages
- No generic equivalent product
- Composition-based patent expires in 2032; pending applications, if granted, may potentially expire in 2037; potentially NCE eligible

KP484

- Prodrug of d-MPH (SDX) with extended release properties
- Potential features and benefits
  - Once-daily dosing
  - Longer duration than other extended release ADHD products
  - Lower abuse potential
- No generic equivalent product
- Composition-based patent expires in 2032; pending applications, if granted, may potentially expire in 2037; potentially NCE eligible

KP415 vs. Selected ER Stimulant Products for ADHD

Product Features[1,2]	KP415	Vyvanse®	Adderall XR®	Focalin XR®	Concerta®
Parent Drug	D-MPH	D-AMPH	Mixed Salts of AMPH	D-MPH	MPH
Technology	ER Prodrug + IR D-MPH	ER Prodrug	ER MAS	ER D-MPH	Osmotic
Dosage Form	Capsule	Capsule & Chewable	ER Capsule	ER Capsule	ER Tablet
Onset	✓✓✓	✗	--	✓✓✓	✗
Duration	✓✓✓	✓✓✓	--	✓	✓
Reduced Drug Liking[3]	✓✓✓	✓✓	✗	✗	✓

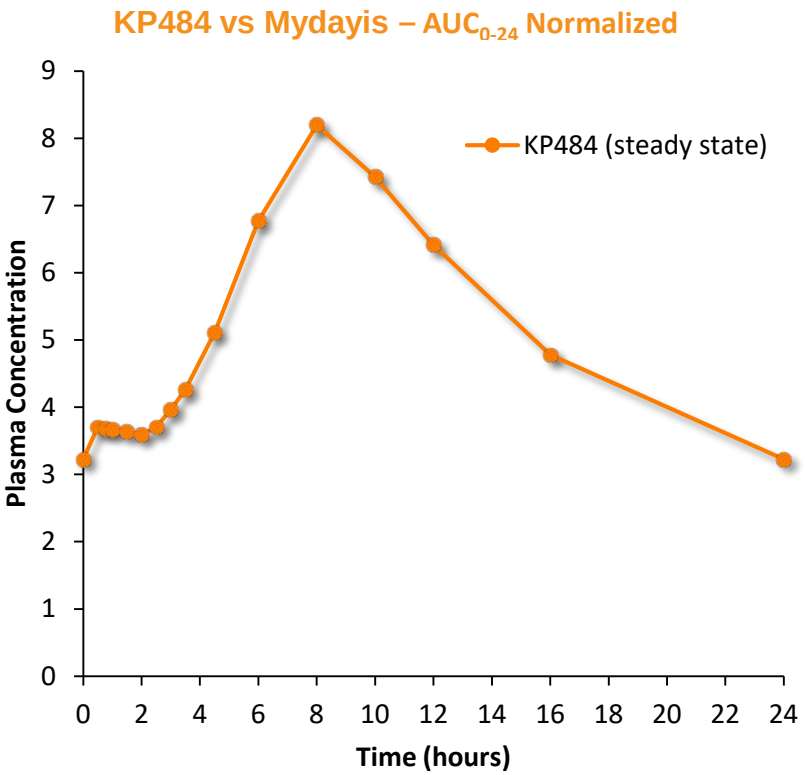
(1) Package insert information for Vyvanse, Adderall XR, Focalin XR and Concerta
(2) Potential product features for KP415, if approved by FDA
(3) For KP415, reduced drug liking for serdexmethylphenidate prodrug component only

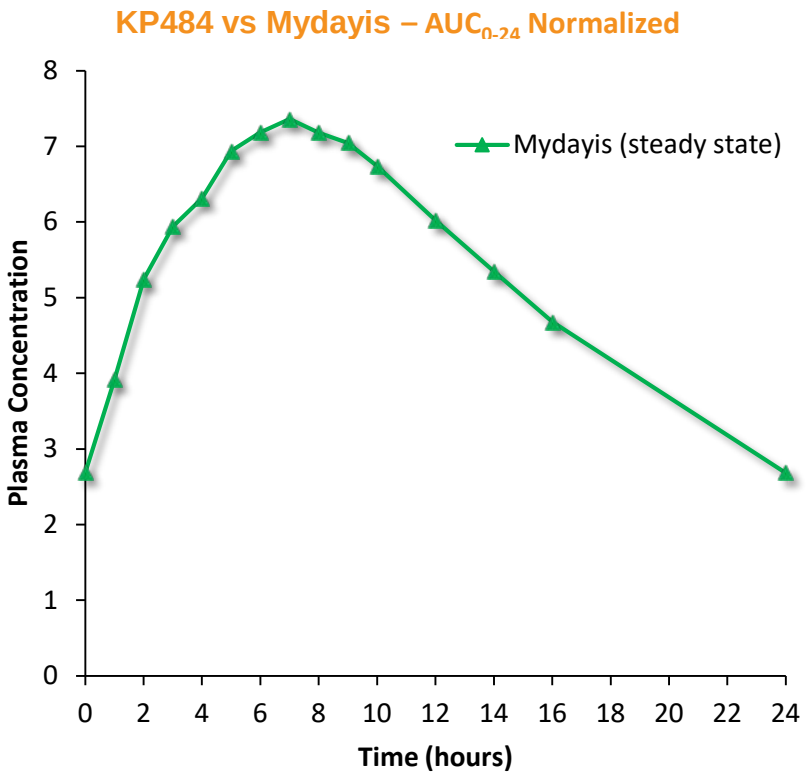
The Adult ADHD Market

- Over 4% of U.S. adults, or approximately 10.5 million adults have ADHD and are now the largest part of the ADHD market, comprising 53% of total TRx[1,2]
- The adult ADHD market has grown at 11% year-over-year vs. 4% for the pediatric ADHD market for the last several years[1]
- Vyvanse®, the ADHD product known for its duration and abuse deterrent features has seen significant growth in the adult market averaging 22% year-over-year growth since 2009[1]
- Shire's Mydayis® was recently approved as a super long-acting product in the amphetamine category (2 to16 hour duration)
- Other potential market opportunities exist within indications where efficacy has been demonstrated by other stimulants or which remain as currently unmet medical needs

1. Symphony Health, PHAST 2011-2018
2. Ronald C. Kessler et al. (April 2006). The Prevalence and Correlates of Adult ADHD in the United States: Results From the National Comorbidity Survey Replication, American Journal of Psychiatry 163(5):71

KP484 Steady State PK vs. Mydayis®[1]





Disclaimer: Mydayis steady-state plasma concentrations were modeled based on Mydayis single-dose data[1] and were normalized so that the mean AUC_{0-24}/C_{max} for d-amphetamine released from Mydayis matches the mean AUC_{0-24}/C_{max} for d-methylphenidate released from KP484. The representation is not intended for predictions or direct comparison of efficacy between the two drugs. Methylphenidate and amphetamine are different stimulants with different potency and MOA.

(1) Spencer TJ, Adler LA, Weisler RH, Youcha SH. Triple-Bead Mixed Amphetamine Salts (SPD465), a Novel, Enhanced Extended-Release Amphetamine Formulation for the Treatment of Adults with ADHD: A Randomized, Double-Blind, Multicenter, Placebo-Controlled Study. *J Clin Psychiatry*. **2008**;69*(9)*:1437-48.

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 Stimulant Abuse in 2016

Stimulant	Abuse Reported In Last 30 Days (% of US Population >12 years)
Prescription Stimulants	1,700,000 (0.63%)
Cocaine[a]	1,900,000 (0.71%)
Methamphetamine[b]	~700,000 (0.25%)
Total:	**4,300,000 (1.59%)**

[a] includes crack cocaine
[b] includes only illicitly manufactured methamphetamine

Source: Substance Abuse and Mental Health Services Administration. (2017). HHS Publication No. SMA 17-5044, NSDUH Series H-52.

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
- May qualify for orphan designation depending on exact indication and target population

KP922

Prodrugs of D-Amphetamine for the Treatment of ADHD

KP922 for the Treatment of ADHD

- Prodrugs of d-amphetamine with various pharmacokinetic profiles
- Product candidates can potentially be tailored to fit various market opportunities and unmet needs including:
  - IR, ER and/or novel pharmacokinetic profiles (blunted C_{max})
  - Co-formulated with various API's (stimulants, non-stimulants)
  - Low abuse potential
- No generic equivalent product
- Potential for long-lived composition of matter patent protection

APADAZ®

FDA Approved for the Short-Term Treatment of Acute Pain

APADAZ® Overview

- First prodrug of hydrocodone (benzhydrocodone) combined with acetaminophen to be approved by the FDA with differentiated properties and long-lived patents
- Commercial license agreement with KVK-Tech, Inc.
  - KemPharm eligible to receive up to an estimated $3.4M in pre-launch payments, milestones and cost reimbursements and additional milestone payments of up to $53M tied to specified net sales levels
  - Net profit share of up to 50% between KemPharm and KVK
  - Most costs and responsibilities shift to KVK; KemPharm remains engaged to support KVK's commercial launch
- The commercial payor category accounted for 1.6 billion HC/APAP tablets, or 41% of a total of 4.0 billion HC/APAP tablets utilized in the U.S. in 2018[1]
- According to MMIT estimates, AG-APADAZ has 70% unrestricted access for Commercial plans[2]
  - Listed at preferred generic for 52% of Commercial plans

1. SHS PHAST: 2018
2. Estimated as of September 5, 2019, according to Managed Markets Insights and Technology; www.formularylookup.com

APADAZ® Next Steps

- We believe broad formulary adoption with preferred generic status represents a key milestone to begin market conversion
- State Medicaid plans have also started to list AG-APADAZ at preferred generic status, including AL and UT
- Actively working with plans to “prefer” AG-APADAZ over HC/APAP
- Outreach to PBM and payor leads by KVK and the managed care access team continues; remain on track for Q4 2019 launch
- KVK is concentrating its planning efforts on manufacturing and distribution for smaller, pilot-scale launches initially, followed by potential national stocking to support broad formulary access
- Based on current formulary access and 2018 utilization data, potential volume of more than 2B tabs addressable for AG-APADAZ

Financial Update

Improved Financial Position

- As of June 30, 2019, total cash[1] was $7.8M
- KP415 license deal provides upfront cash of $10M immediately, with next pre-approval milestone at KP415 NDA acceptance
- R&D spending expected to decline significantly going forward; KP415 program is substantially complete, with remaining costs shifting to partner
- Q2 2019 reduction in workforce (30%), combined with other G&A spending reductions, reduces overall cash burn rate; expect positive impact beginning in Q3 2019
- Focus on debt reduction/restructuring over last 18 months, including recent Deerfield exchange agreement
  - If all optional exchanges are made, total debt will be reduced by 45% to $53.4M as soon as May 2020, down from $96.3M in May 2018
- If KP415 license milestones are met, would add significant non-dilutive capital through KP415 approval and beyond

1 - Includes cash, cash equivalents and restricted cash

Conclusion

KemPharm Next Steps

- KP415
  - Work with GPC on review and NDA submission, expect to file in 2019
  - PDUFA date in 2020 based on anticipated NDA submission date
- KP484
  - Work with GPC to review and finalize the product development plan, expect to fully commence development following KP415's NDA filing
- APADAZ®
  - Work with KVK to launch APADAZ in Q4 2019
- KP879
  - File IND for KP879 in Q4 2019
- KP922
  - Advance one or more product candidates to a potential IND filing in 2020
- Continue our efforts to reduce debt and cash burn
- Discovery and Research efforts ongoing in multiple therapeutic areas as we continue to develop more pipeline opportunities

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KemPharm

Leveraging our LAT™ Prodrug Technology to Create Long-Term Value

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