KemPharm

ADHD Prodrug Pipeline Update

June 28, 2017

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This presentation contains forward-looking statements, including statements about 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 the expected features and characteristics of our product candidates, the clinical utility of our product candidates, the anticipated timelines for any submissions to the FDA or the availability of clinical trial results 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 Annual Report on Form 10-K filed with the SEC on March 10, 2017, 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.

This presentation also contains 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.

Extending ADHD Prodrug Pipeline & Value Potential

- ✓ **Completed End-of-Phase 1 Meeting with FDA for KP415**
  - ADHD treatment focused on addressing unmet need with a faster onset of efficacy with a longer total duration than current methylphenidate treatment options
  - KP415 clinical program affirmed by FDA
  - Should data be favorable, clinical and PK Studies may be sufficient for as early as late 2018 NDA submission
- ✓ **Announced Development of New ADHD Product Candidate – KP484**
  - ADHD product candidate focused on addressing unmet need with potentially longer duration than other current methylphenidate treatments, including KP415
  - KemPharm intends to file IND for KP484 as early as 3Q 2017; NDA submission targeted as early as 2019
  - Development pathway may benefit from KP415 research

ADHD Prodrug Product Pipeline – Addressing Unmet Needs

KP415 – ADHD Product Candidate for Fast Onset and Longer Total Duration

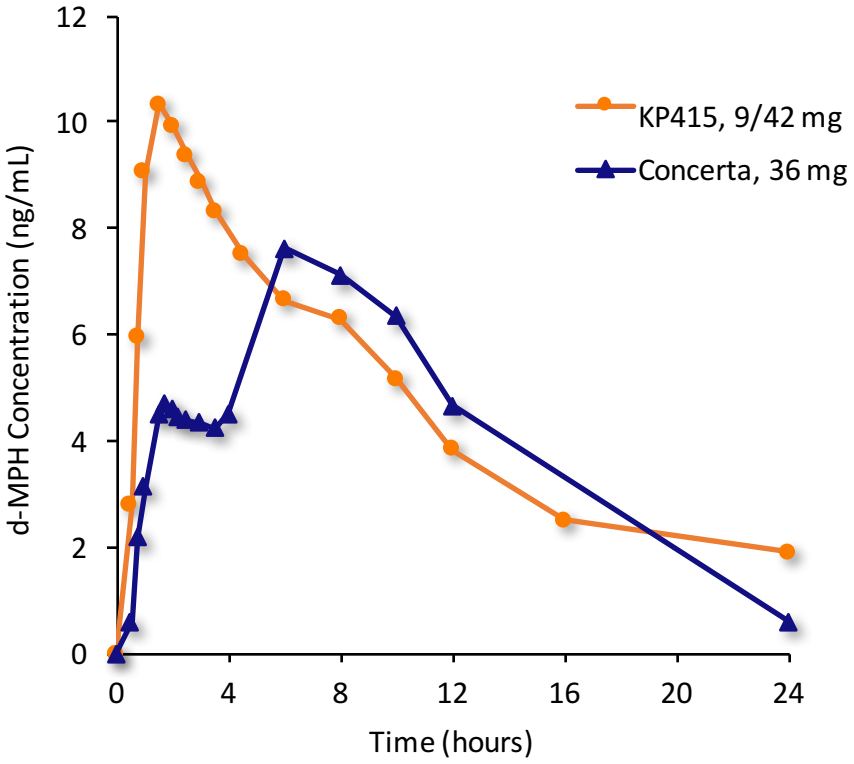
- Extended release prodrug of d-threo-methylphenidate (d-MPH)
- Phase 1 PoC data complete
- Additional PK studies underway; final PK study data anticipated in 2H 2017 through early 2018
- Human abuse liability clinical data by year end (IV) as well as in 2018 (oral and IN)
- Pivotal efficacy trial expected to begin in 2H 2017; data expected 1H 2018
- KP415 NDA filing expected as early as late 2018

KP484 – ADHD Product Candidate with Super Extended Release Properties

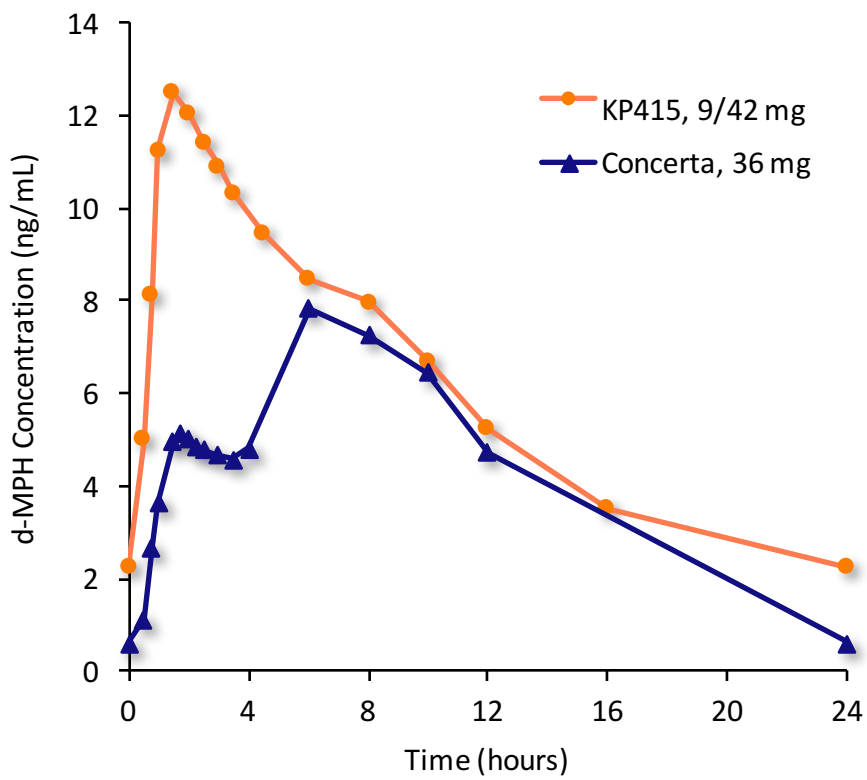
- Newly developed, super extended release prodrug of d-MPH
- Initial data suggest long acting characteristics similar to Shire's MYDAYIS™ (amphetamine-based)
- Human abuse liability clinical data as early as year end (IV) and early 2018 (oral and IN)
- IND expected to be filed as early as 3Q 2017
- Clinical program initiated under KP415 IND; expect benefit from KP415's development program
- Potential NDA as early as 2019

Predicted Oral PK Data – KP415

Commercial KP415 – Predicted PK, Single Dose



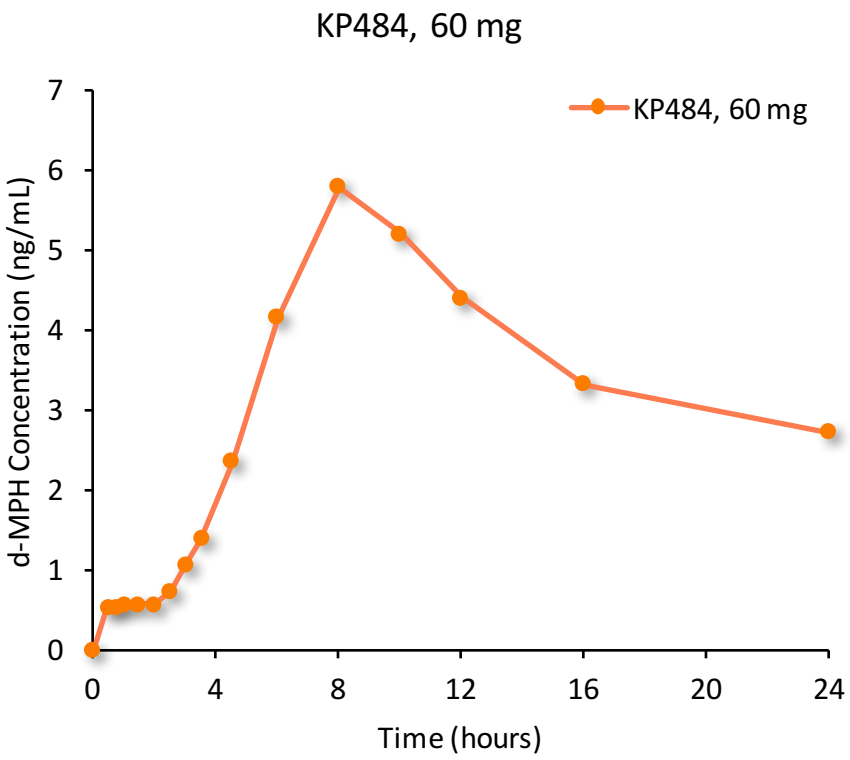
Commercial KP415 – Predicted Oral PK, Steady State



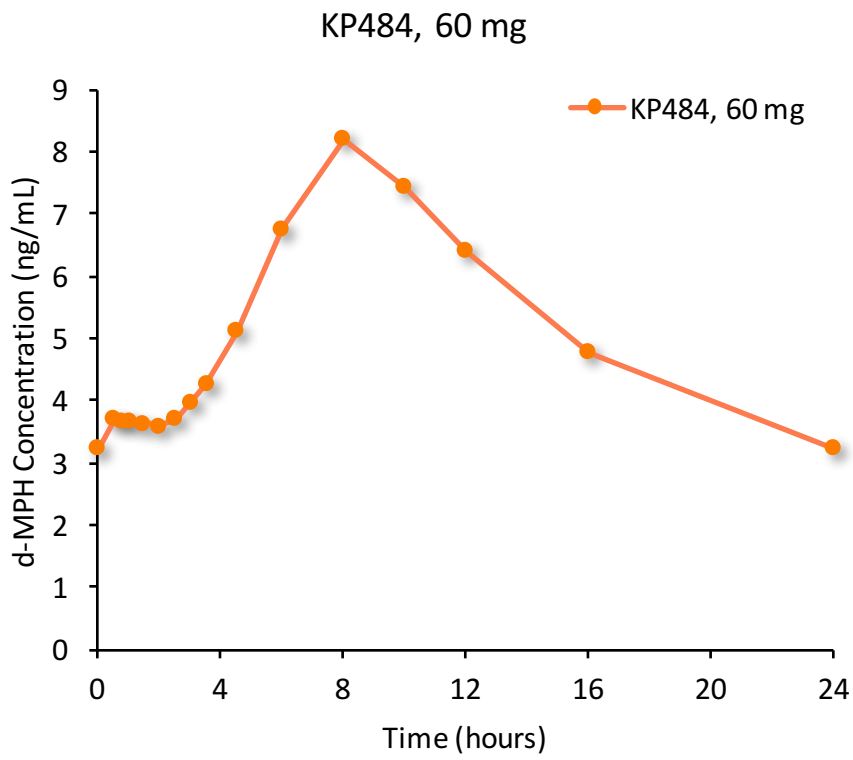
Note: Steady-state plasma concentrations were modeled based on single-dose data.

Oral Pharmacokinetic Data – KP484

KP484 – Oral PK, Single Dose



KP484 – Predicted Oral PK, Steady State



Note: Steady-state plasma concentrations were modeled based on single-dose data.

Completed Clinical Studies of KP484

- KP484 Clinical Data to Date
  - Single dose oral bioavailability and dose proportionality of 20, 40, and 60 mg in capsules
  - Urinary excretion study following single oral dose of 6 and 60 mg administered as oral liquids
  - Effect of food on the oral bioavailability and pharmacokinetics with a 60 mg capsule

ADHD and ER Methylphenidate Market

- ~$13 billion ADHD market with prescriptions growing at >5% year-over-year
- Methylphenidate accounted for approximately 19.8 million TRx's and $3.8 billion in sales in 2016
- KemPharm believes ADHD key opinion leaders have significant interest in an ER methylphenidate product with:
  - Earlier onset (KP415)
  - Improved duration of action (KP415 & KP484)
  - Abuse-deterrent properties/Lower abuse potential (KP415 & KP484)
- Branded products are being pressured by patent expirations
  - Vyvanse™ is the branded market share leader and loses patent exclusivity in 2024
  - Concerta™, Adderall ™, Focalin ™ are all brands which are off patent

Source: Symphony Health, PHAST 2016

KP415: ADHD Market Dynamics

- Although the ADHD market has become more genericized, many generics are priced closely to their branded comparator's. In 2016, the branded ADHD market was ~$6.4B(1)
  - >95% of these branded products are Extended Release (1)
- In the prior 4 years, there have been seven (7) product launches based on delivery mechanisms alone; if approved, KP415 has the potential to be one of the first differentiated products launched in the ADHD market in some time
- Market research cites that 60% of prescribers saw KP415's potential duration of action as a key advantage. This was ranked in front of abuse potential (52%) and early onset of action (43%)
- Market research cites that prescribers estimate that ~60% of the time, methylphenidate is given as the preferred first line of therapy for children under the age of 13

(1) Source: Symphony Health, PHAST 2016

KP484: Adult ADHD Market Dynamics

- It is estimated that 4.4% of adults in the U.S. have ADHD(3)
- When applied to the full U.S. adult population aged 18 and over, there are potentially 10.5 million adults in the U.S. with ADHD(2)
- If approved, KP484 would launch into the high growth adult ADHD market.
  - Over the last seven (7) years the adult market has grown at 11% YoY vs 4% for the pediatric market(3)
  - Adults are now the largest part of the ADHD market, comprising 53% of total TRx. In 2009, adults contributed 42% of total TRx(4)
  - Despite the rapid growth in the adult market, manufacturers continue to address unmet needs for children. The last 7 products launched in the ADHD space have been pediatric focused(5)
  - Vyvanse™, the product known for its duration & ADF has seen significant growth in the adult market averaging 22% YoY growth since 2009(6)

Adult sales of Vyvanse™ are trending to surpass Pediatric sales in 2017

KP484: Adult ADHD Market Dynamics, cont.

- There is an estimated 38MM patients on ADHD medications, of which ~9% are taking an ER+ER and 10% are ER+IR(7)
  - A once daily dose could eliminate the need for multiple drugs, potentially improving adherence & reducing patient OOP costs (single copay)
- It is estimated that Shire's SXR amphetamine-based MYDAYIS™ will have >$1.5B in sales over the next six (6) years, with sales projected at $416MM in 2022(8)
  - This launch may help build awareness for once a day ADHD treatments
- The most frequently mentioned unmet need in the ADHD space is duration of action (per approximately two-thirds of prescribers surveyed)
- Per market research, nearly one third of prescribers (31%) are very or extremely concerned with the potential abuse/misuse of stimulant based therapies

KP484: Other Potential Indications

KP484 could provide the potential for other indications that have either been demonstrated by other stimulants or are unmet medical needs including:

1. Binge Eating Disorder (Vyvanse™)
2. Excessive Daytime Sleepiness (various modafinil products)
3. Stimulant dependence
4. Adjunctive therapy for other co-morbid CNS diseases

KemPharm Clinical Product Pipeline

Category	Product Candidate	Parent Drug	Development Status	Next Milestone	Potential NDA Submission
ADHD (Pediatric)	KP415	Methylphenidate (ER)	Clinical	PK + Efficacy Data	2018
ADHD (Adult)	KP484	Methylphenidate (ER)	Clinical	PK + Efficacy Data	2019
PAIN	KP201/IR	Hydrocodone	Clinical	IN HAL Data	2018 with Priority Review
	KP511/ER	Hydromorphone	Clinical	POC in ER Formulation	2019 with Priority Review
	KP511/IR	Hydromorphone	Clinical	HAL and BE Data	2019 with Priority Review

Expected ADHD Pipeline Milestones

	Product	Event
2017	KP415	Report Additional PK Data (2H)
	KP415	Initiate Pivotal Efficacy Trial (2H)
	KP484	IND Filing (3Q)
	KP415 / KP484	IV Human Abuse Liability Data (2H)
2018	KP415	Pivotal Efficacy Trial Results (1H)
	KP484	Initiate Efficacy Studies
	KP415 / KP484	Oral and IN HAL Data
	KP415	NDA Submission
2019	KP484	Clinical Trial Program Execution / Completion
	KP484	NDA Submission

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KP484 Market Dynamics: Sources

(1) Symphony Health, PHAST 2011-2016
(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
(3) Symphony Health, PHAST 2011-2016
(4) Symphony Health, PHAST 2011-2016
(5) Data on File
(6) Symphony Health, PHAST 2011-2016
(7) Symphony Health Transactional Data, Jun-Aug 2015
(8) Evaluate Pharma, SHP465 Worldwide Report 2017