KemPharm

Canaccord Genuity 37th Annual Growth Conference

August 10, 2017

Cautionary Note Regarding Presentation Information

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 our ability to use the 505(b)(2) pathway and expedited FDA review, the clinical utility of 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 May 22, 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.

KemPharm Overview

- Specialty pharmaceutical company discovering and developing novel **prodrugs**
- Leveraging our **LAT™ Platform Technology** to improve the attributes of approved drugs in large markets
- Building a pipeline of **product candidates** for ADHD, pain and CNS disorders
- Potentially utilizing FDA's **505(b)(2) pathway** to reduce risk and expense
- Generating long-lived **composition-of-matter** patent protection

LAT™ (Ligand Activated Therapy) Platform Technology

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

- Proprietary to KemPharm and is **applicable across therapeutic areas**
- Amenable to **both immediate and extended release formulations**

KemPharm Clinical Product Pipeline

Category	Product Candidate	Parent Drug	Development Status	Next Milestone	Potential NDA Submission
ADHD	KP415	Methylphenidate (ER)	Clinical	PK + Efficacy Data	2018
ADHD	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

Attention-Deficit/Hyperactivity Disorder:

KP415 and KP484
For the Treatment of ADHD

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

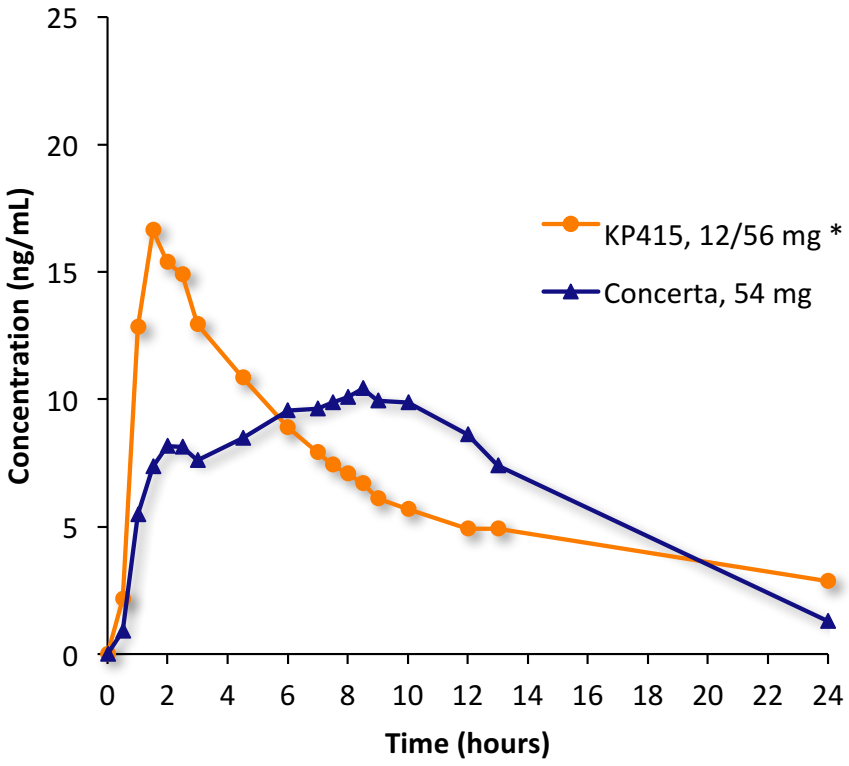
- In 2016, the branded ADHD market was ~$6.4B and more than 95% of these branded products are extended release[1]
- ADHD market has become more genericized, but many generics are priced closely to their branded comparator
- Recent ADHD new product launches have been based on delivery mechanisms alone; if approved, KP415 has the potential to be one of the first differentiated products launched into the ADHD market in some time
- Market research indicates prescribers see the following KP415 key advantages
  - Duration of action (60%)
  - Lower abuse potential (52%)
  - Early onset of action (43%)
- Market research indicates that prescribers estimate that methylphenidate is given as the preferred first line of therapy for children under the age of 13 approximately 60% of the time

KP415 Product Overview

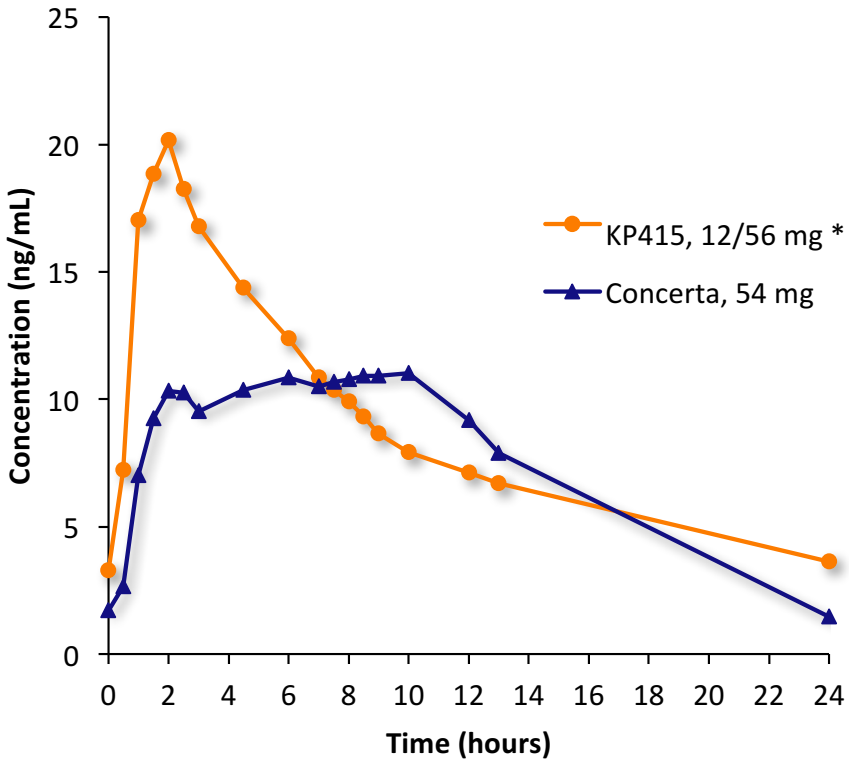
- Prodrug of d-methylphenidate (MPH) with intrinsic extended release properties, co-formulated with immediate release d-methylphenidate
- Potential KP415 features and benefits
  - Early onset of action
  - Potential longer total duration than current MPH therapies
  - Active metabolism may offer more predictable therapeutic effect
  - Lower abuse potential
  - Patient-friendly dosage form
    - Small capsule size (same as Vyvanse), easily swallowed
    - May be opened and contents sprinkled on food or mixed into liquids for easier ingestion
- No generic equivalent product
- Composition-based patent expires in 2032, and is potentially NCE eligible

KP415 – Single and Multiple Oral Dose PK

Commercial KP415 –Single Dose PK



Commercial KP415 – Steady State PK

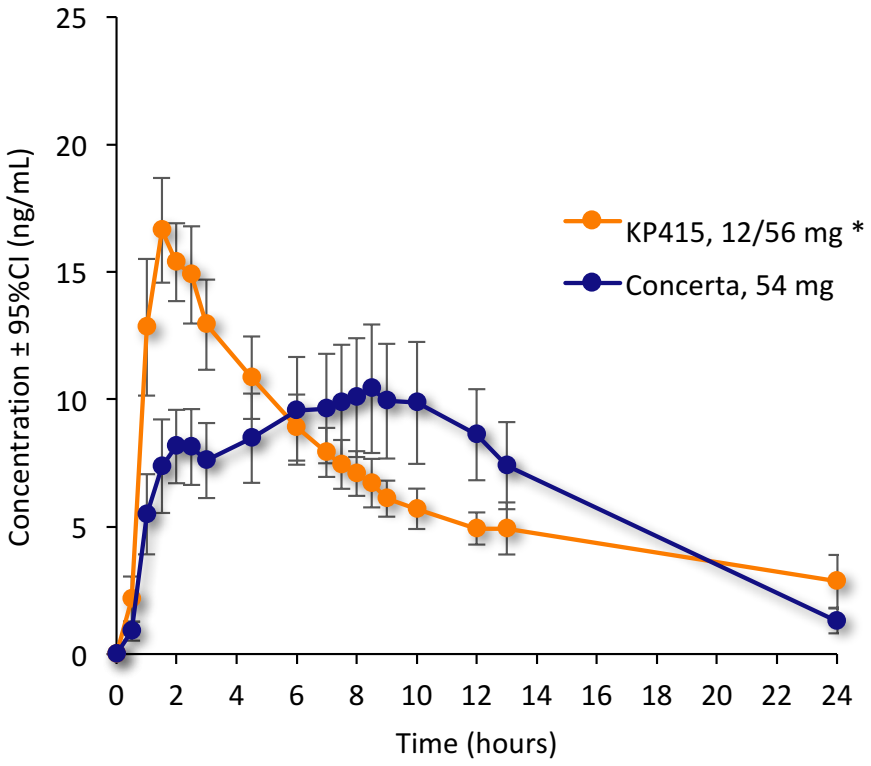


Note: Steady-state plasma concentrations collected after 7 days of once-per-day dosing.

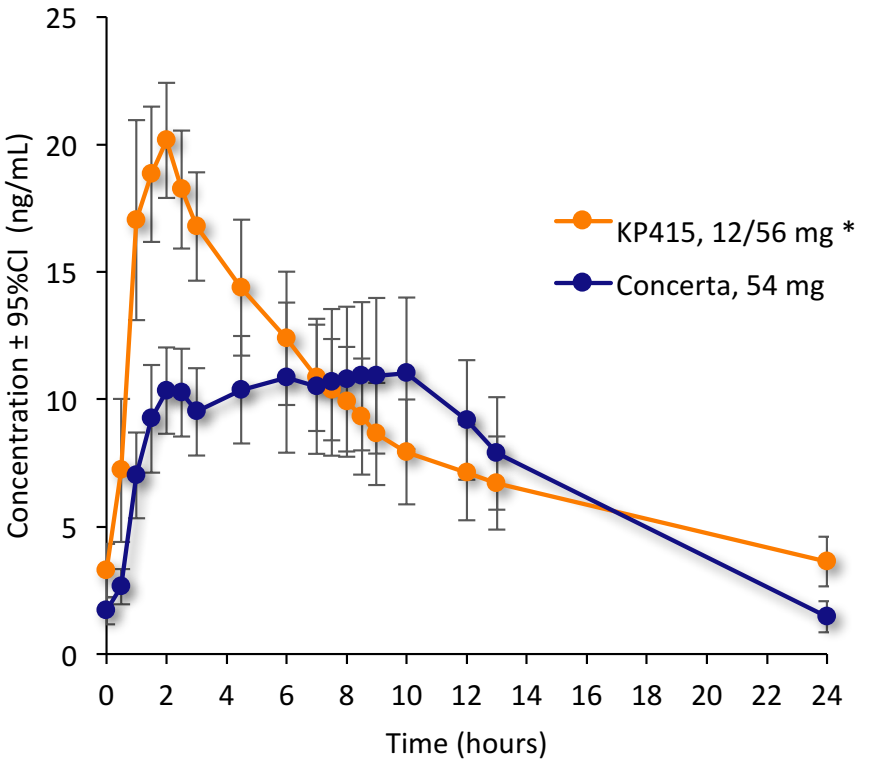
* Two subjects of the KP415, 12/56 mg treatment arm who appeared to be slow metabolizers of d-MPH were excluded from the analysis.

KP415 – Single and Multiple Oral Dose PK

Commercial KP415 –Single Dose PK



Commercial KP415 – Steady State PK



Note: Steady-state plasma concentrations collected after 7 days of once-per-day dosing.

* Two subjects of the KP415, 12/56 mg treatment arm who appeared to be slow metabolizers of d-MPH were excluded from the analysis.

KP484: Adult ADHD Market Dynamics

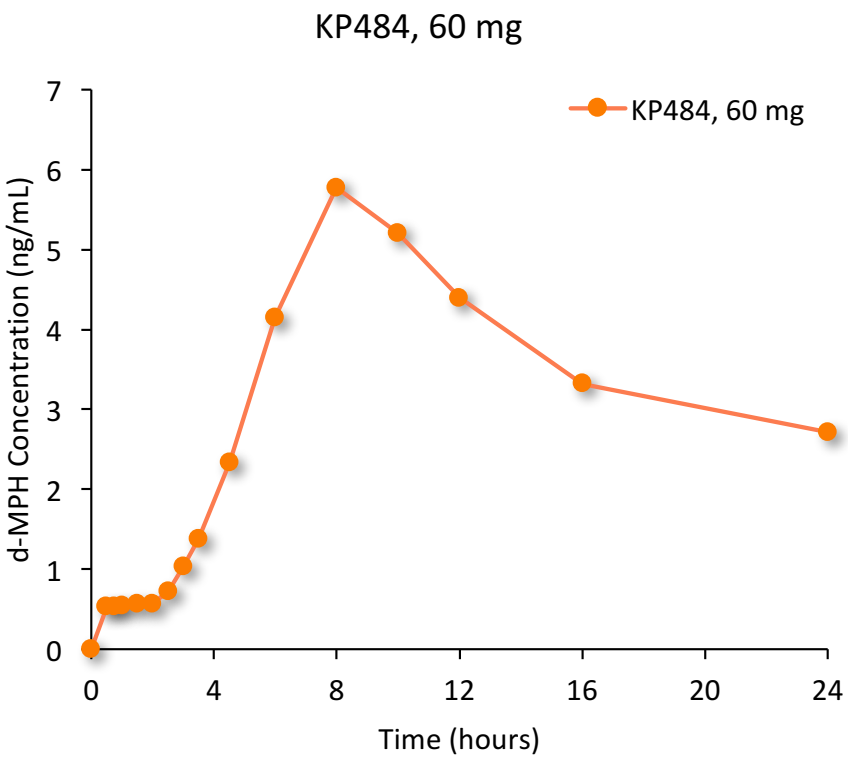
- Over 4% of U.S. adults, or approximately 10.5 million adults have ADHD[1,2]
- If approved, KP484 would launch into the high growth adult ADHD market
  - The adult ADHD market has grown at 11% YoY vs. 4% for the pediatric ADHD market for the last several years[1]
  - Adults are now the largest part of the ADHD market, comprising 53% of total TRx[1]
  - Despite the rapid growth in the adult market, the last 7 new ADHD products launched have been pediatric focused
  - Vyvanse™, the ADHD product known for its duration and abuse deterrent features has seen significant growth in the adult market averaging 22% YoY growth since 2009[1]
  - Shire's Mydayis™ was recently approved as a super long acting AXR in the amphetamine space (4-16 hour duration)
- KP484 could also provide the potential for other indications that have either been demonstrated by other stimulants or are currently unmet medical needs

KP484 Product Overview

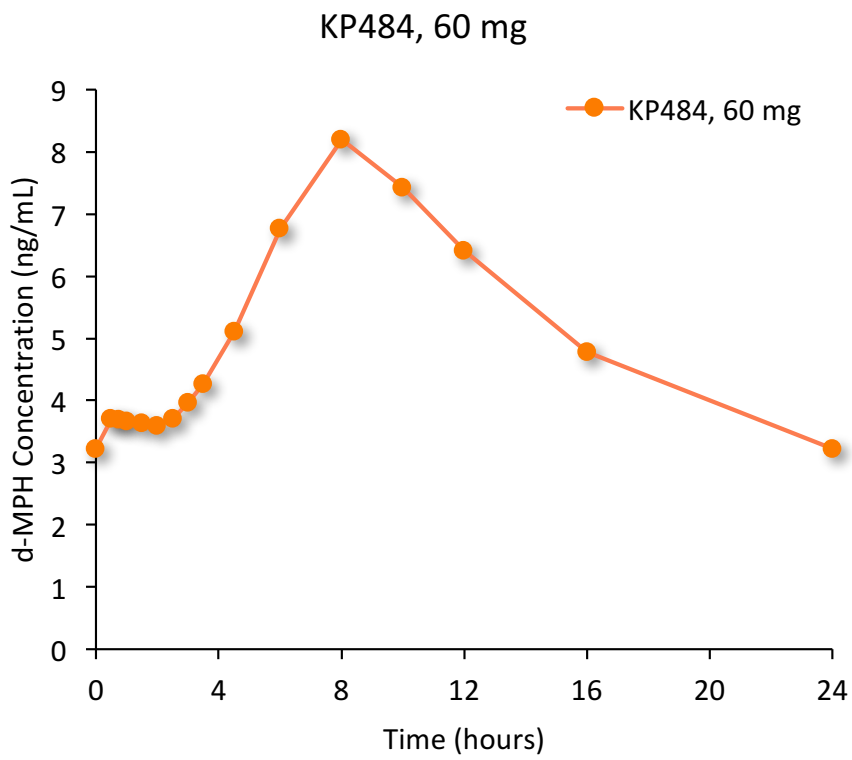
- Prodrug of d-methylphenidate with extended release properties
- Potential KP484 features and benefits
  - True once-daily dosing with potentially up to 16 hour duration
  - Potentially longer duration than other super-extended release ADHD products
  - Active metabolism may offer more predictable therapeutic effect
  - Lower abuse potential
  - Patient-friendly dosage form
    - Small capsule size (same as Vyvanse), easily swallowed
    - May be opened and contents sprinkled on food or mixed into liquids for easier ingestion
- No generic equivalent product
- Composition-based patent expires in 2032, and is potentially NCE eligible

KP484: Single and Predicted Multiple Oral Dose PK

KP484 – Oral PK, Single Dose

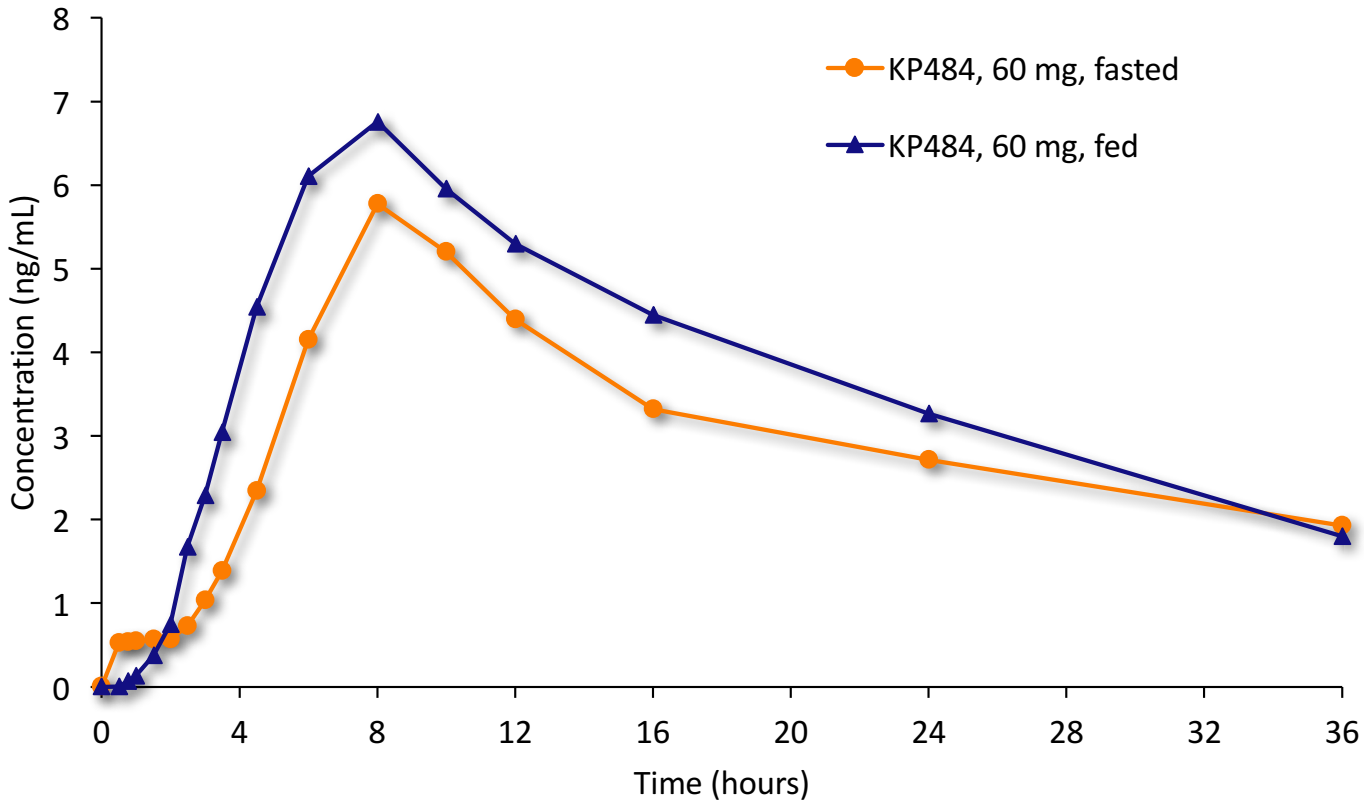


KP484 – Predicted Oral PK, Steady State



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

KP484: Effect of Food on Oral PK



Note: Subjects in the fed treatment arm received a single dose of KP484, 60 mg within 20 minutes following a standardized breakfast.

KP415 Clinical Update and Development Timeline

- Completed End-of-Phase 1 Meeting on June 14, 2017
- FDA raised no objections to KP415 clinical program
  - Single efficacy study
- Data from multiple PK studies support PK profile of the prodrug and KP415 in both single dose and multiple dose settings which replicates current therapy and efficacy study setting
  - No effect with a normal breakfast as well as dose proportionality with our anticipated doses
- Intravenous (IV) Human Abuse Liability (HAL) data anticipated by year-end 2017, with oral and intranasal (IN) HAL data anticipated in 2018
- KP415 pivotal efficacy studies anticipated to commence in 2H 2017, with final data expected in 1H 2018
- KP415 NDA anticipated to be filed as early as late 2018

KP484 Clinical Update and Development Timeline

- KP484 IND filing anticipated in 3Q 2017, but clinical program already initiated under KP415's IND

- KP484 and KP415 are each expected to benefit from each other's development program, with KP484/KP415 clinical studies completed to date including:
  - Single and multiple dose study with KP415 at (12/56 mg)
  - Single dose oral bioavailability study (20, 40, and 60 mgs)
  - Single dose urinary excretion study (6 and 60 mgs)
  - Food effect study (60 mg)

- Intravenous (IV) Human Abuse Liability (HAL) data anticipated by year-end 2017, with oral and intranasal (IN) HAL data anticipated in 2018

- KP484 NDA anticipated to be filed as early as 2019

KemPharm Expected ADHD 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

Q1 2017 Financial Update

- Q2 2017 results to be announced today, August 10, 2017 at 4:30pm ET
- Total cash[1] of $72.4M as of March 31, 2017
  - Used $9.7M of cash during Q1 2017
- Q1 2017 net loss of $16.3M, or $1.11 per basic and diluted share vs. Q1 2016 net loss of $2.9M, or $0.20 per basic and diluted share
  - Net loss for Q1 2017 increased primarily due to non-cash fair value adjustments to our derivative and warrant liabilities, which shifted from non-cash income of $10.3 million in Q1 2016 to a non-cash loss of $7.2 million in Q1 2017
  - Loss from operations increased to $7.4M in Q1 2017, as compared to $7.0M in Q1 2016, which was driven by an increase in R&D spending of $0.9M on the development programs for KP415, KP201/IR and KP511, offset by a reduction in G&A of $0.5M
- 14,646,982 common shares outstanding at March 31, 2017

1.Includes cash, cash equivalents, restricted cash, marketable securities, trade date receivables and long-term investments

KemPharm

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Slides 8 and 12: KP415 and KP484 Market Data 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