KemPharm

KP415.E01 Efficacy Trial Results

July 9, 2018

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KP415.E01 Efficacy Trial Results Call Participants

- **Travis Mickle, Ph.D.** – President & Chief Executive Officer
- **R. LaDuane Clifton, CPA** – Chief Financial Officer, Secretary & Treasurer
- **Gordon K. "Rusty" Johnson** – Chief Business Officer
- **Dan Cohen, M.A.L.S.** – EVP, Government & Public Relations

KP415 Product Overview

- Prodrug of d-methylphenidate (MPH) with 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
- Granted composition-based patent expires in 2032, pending applications potentially would expire in 2037; potentially NCE eligible

KP415.E01 Efficacy Trial Results

- Met primary endpoint of the trial: mean difference in SKAMP-C score change from baseline across all post-dose time points ($p<0.001$)
- Trial results, based on a prespecified analysis of SKAMP-C scores using the Visit 5 pre-dose value as baseline, support a 1 hour post-dose onset of action and a duration of efficacy of 10 hours post-dose
  - A period effect occurred between Visit 5 and Visit 6 where observed placebo SCAMP-C scores changed unexpectedly from Visit 5 to Visit 6
- We believe our post hoc analysis of SKAMP-C scores based on using the Visit 6 pre-dose value as baseline supports a 30 minute post-dose onset of action and a duration of efficacy of 13 hours post-dose
- Secondary endpoints supportive of a 30 minute post-dose onset of action and a duration of efficacy of 13 hours post-dose include:
  - PERMP (Total Score), PERMP-A and PERMP-C change from baseline
- Additional secondary endpoints appear supportive of overall efficacy

KP415.E01 Primary Endpoint Result

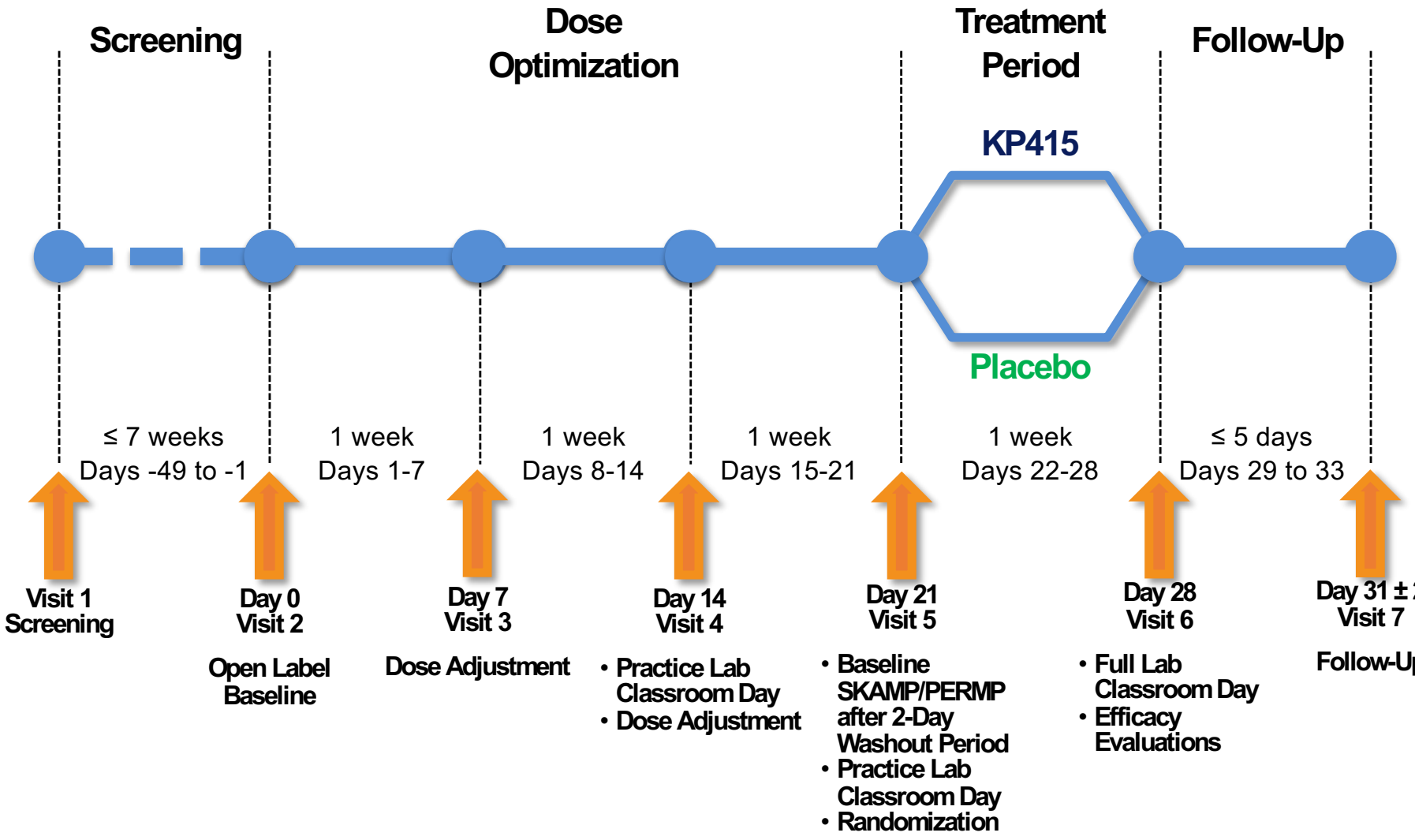
- KP415.E01 met the primary endpoint of mean difference in change from baseline (pre-dose Visit 5) across all post-dose time points for the SKAMP-C score ($p<0.001$)

Change in SKAMP-C from Pre-dose Visit 5						
	LS Mean (SE)		Difference in LS mean (SE)	95% Confidence Interval		
Statistical Measure	KP415	Placebo	KP415 – Placebo	KP415 – Placebo		p-Value
Mean difference in change from baseline across all post-dose time-points	-4.87 (0.62)	0.54 (0.70)	-5.41 (0.87)	-7.10	-3.71	<0.001

KP415.E01 Efficacy Trial Overview

- Double-blind, placebo-controlled, randomized, parallel, analog classroom trial
- Children 6 to 12 years with ADHD (150 completers)
- 5 U.S. trial sites, 2-3 cohorts each; 5-18 subjects per cohort/per site
- 3-week open-label KP415 dose optimization period ending with 2-day drug washout period
- On Visit 5 (Day 21), baseline SKAMP-C scores were collected at pre-dose, patients were given their last open-label dose and then they were randomly assigned to either placebo or their optimized dose of KP415 once daily in the morning for 1 week
- On Visit 6 (Day 28), SKAMP-C scores were collected at pre-dose and post-dose efficacy assessments were taken at 0.5, 1, 2, 4, 8, 10, 12 and 13 hours

KP415.E01 Efficacy Trial : Design Schematic

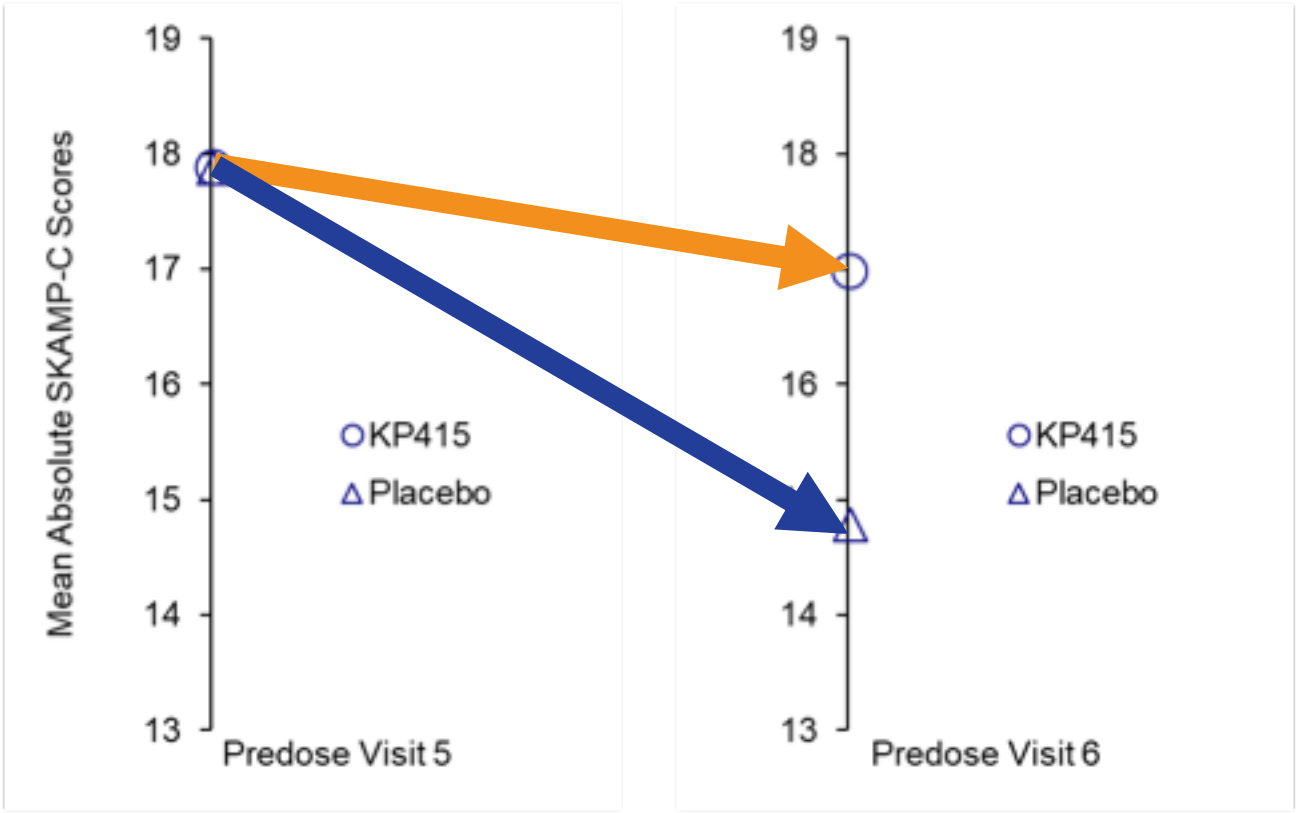


Selection of Visit 5 as Pre-Dose Baseline

- The Visit 5 pre-dose SKAMP-C scores were pre-specified in the Statistical Analysis Plan (SAP) as the baseline
- Visit 5 was selected due to concerns related to a potential carryover effect related to the steady-state pharmacokinetic profile of KP415
- No KP415 carryover effect, as evidenced by pre-dose SKAMP-C and PERMP scores taken on Visit 6, was ultimately observed
- However, the Placebo group did have baseline changes from Visit 5 to Visit 6 that were not anticipated
- Using Visit 5 as pre-dose baseline did not correct for Visit 6 pre-dose differences between Active and Placebo groups

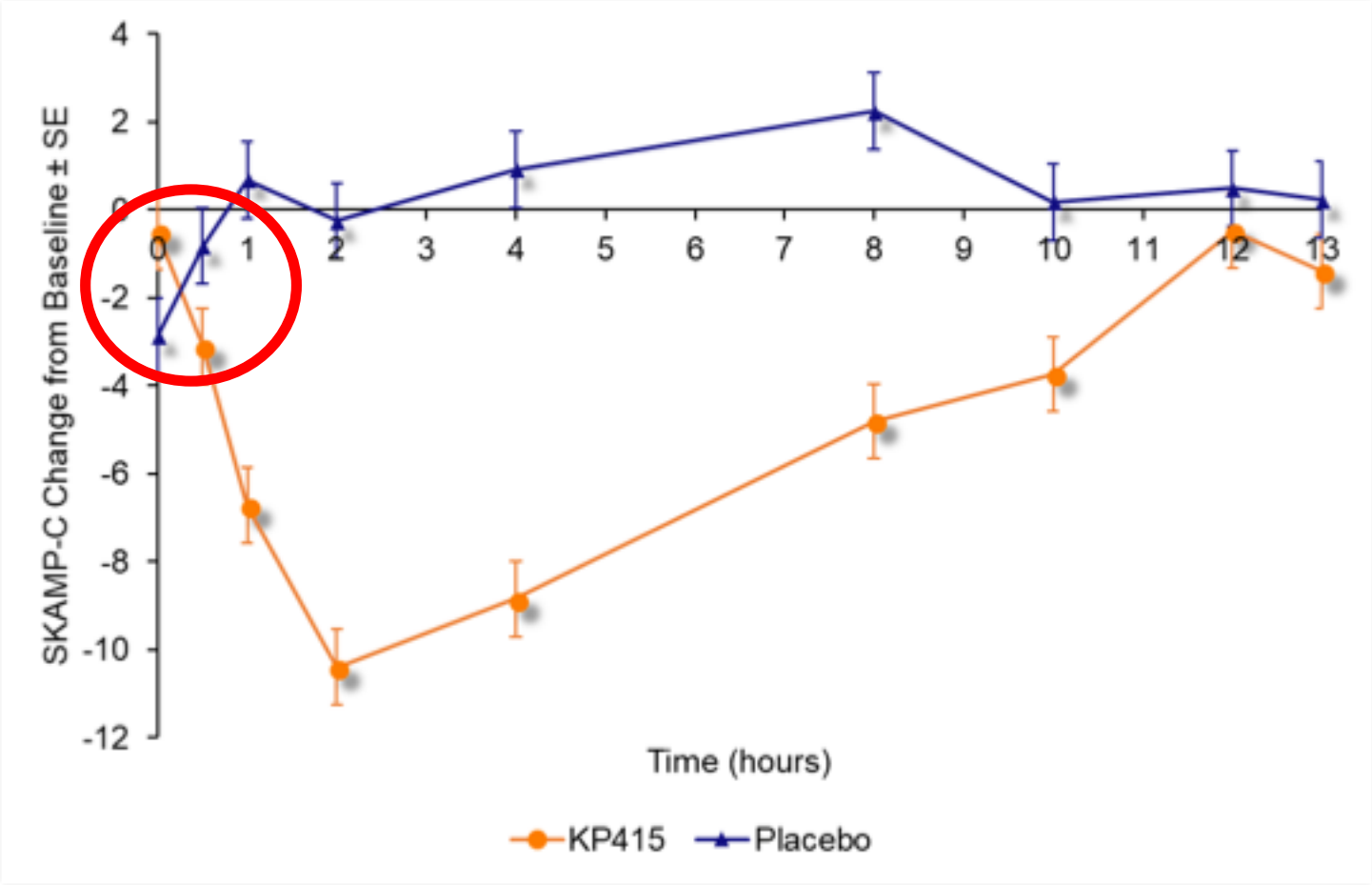
Absolute SKAMP-C Scores for Visit 5 vs. Visit 6 Pre-Dose Statistical Baselines

(Note: This slide was added subsequent to the July 9, 2018 investor presentation)



Visit 5: both KP415 and Placebo subjects had taken KP415 for 3 weeks followed by a 2-day washout. After baseline assessment, all subjects received a KP415 dose.
Visit 6: KP415 subjects had taken KP415 for 1 week and Placebo subjects had taken Placebo for 1 week. After baseline assessment, the last dose of either KP415 or placebo was administered.

SKAMP-C Change on Visit 6 Using Visit 5 Pre-Dose Baseline



As per pre-specified SAP. LS means shown. Statistical model includes predose Visit 5 as covariate.

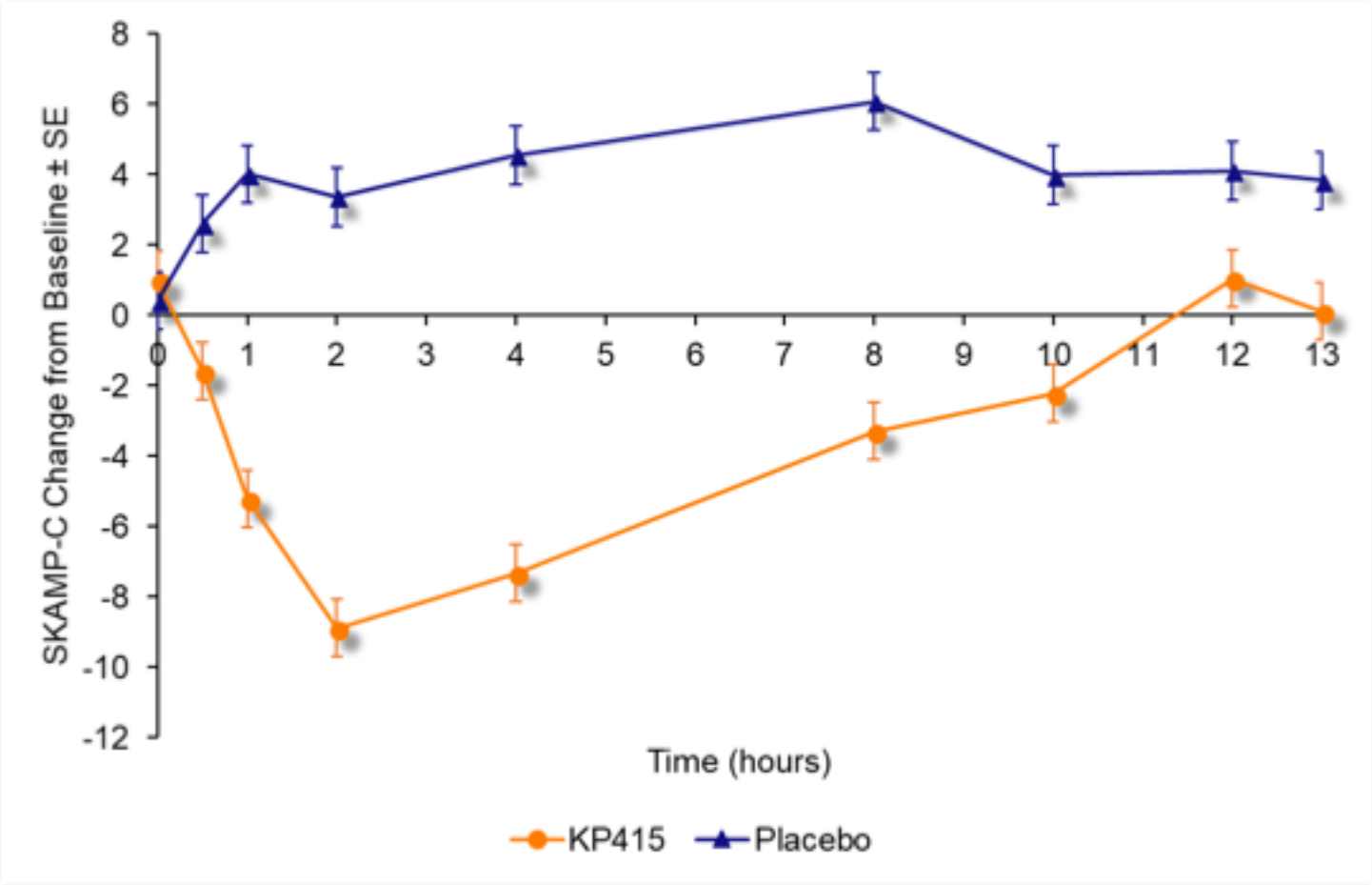
Selection of Visit 6 as Pre-Dose Baseline

- Using the Visit 6 pre-dose SKAMP-C scores as the baseline corrects for any pre-dose differences between the Active and Placebo groups on the efficacy assessment day

- Using Visit 6 (full classroom day) pre-dose SKAMP-C scores as the baseline has been acceptable in the past with efficacy studies conducted for other FDA approved ADHD products, the results of which appear in their respective labels

- Several precedents exist for ADHD-related trials where FDA has considered and/or requested alternative statistical analyses due to unexpected study influences that generated bias in either the Active or Placebo groups, including changes seen in Placebo and Active groups from period to period

SKAMP-C Change on Visit 6 Using Visit 6 Pre-Dose Baseline



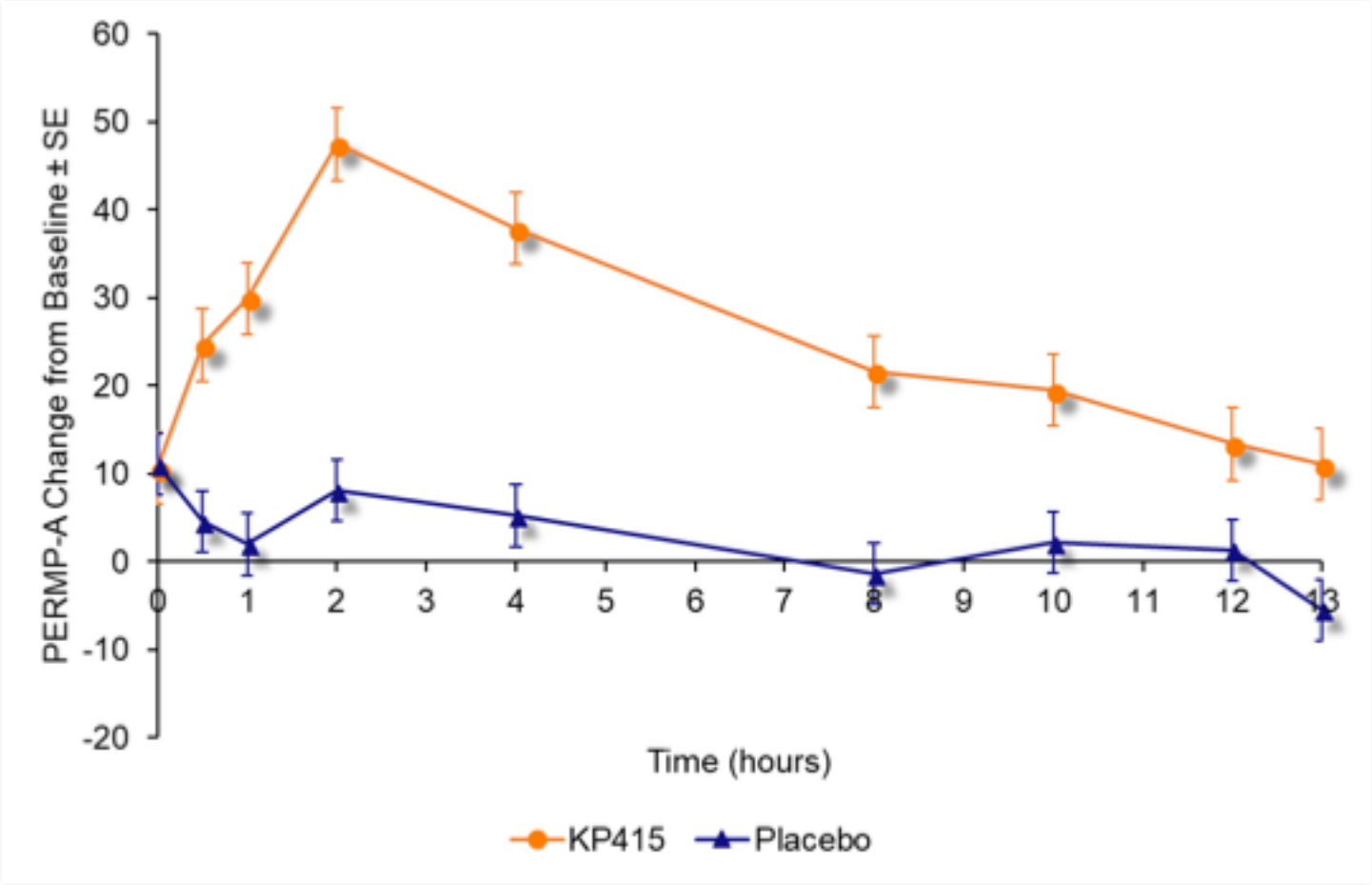
LS means shown. Statistical model also includes predose Visit 6 as covariate.

Differences in SKAMP-C Change Between KP415 and Placebo on Visit 6 Using Visit 5 vs. Visit 6 Pre-Dose Values as Baseline

Time	SKAMP-C Change at Visit 6 from Baseline			
	Baseline = predose Visit 5 (prespecified)*		Baseline = predose Visit 6 (post hoc)*	
	Difference in LS mean (SE) KP415 – Placebo	p-Value	Difference in LS mean (SE) KP415 – Placebo	p-Value
Predose	2.37 (1.18)	0.044	0.599 (1.14)	0.600
0.5 hours postdose	-2.28 (1.18)	0.053	-4.19 (1.14)	<0.001
1 hours postdose	-7.40 (1.18)	<0.001	-9.22 (1.14)	<0.001
2 hours postdose	-10.14 (1.18)	<0.001	-12.25 (1.14)	<0.001
4 hours postdose	-9.76 (1.18)	<0.001	-11.88 (1.14)	<0.001
8 hours postdose	-7.05 (1.18)	<0.001	-9.37 (1.14)	<0.001
10 hours postdose	-3.91 (1.18)	<0.001	-6.20 (1.14)	<0.001
12 hours postdose	-0.96 (1.18)	0.412	-3.07 (1.14)	0.007
13 hours postdose	-1.63 (1.18)	0.167	-3.71 (1.14)	0.001

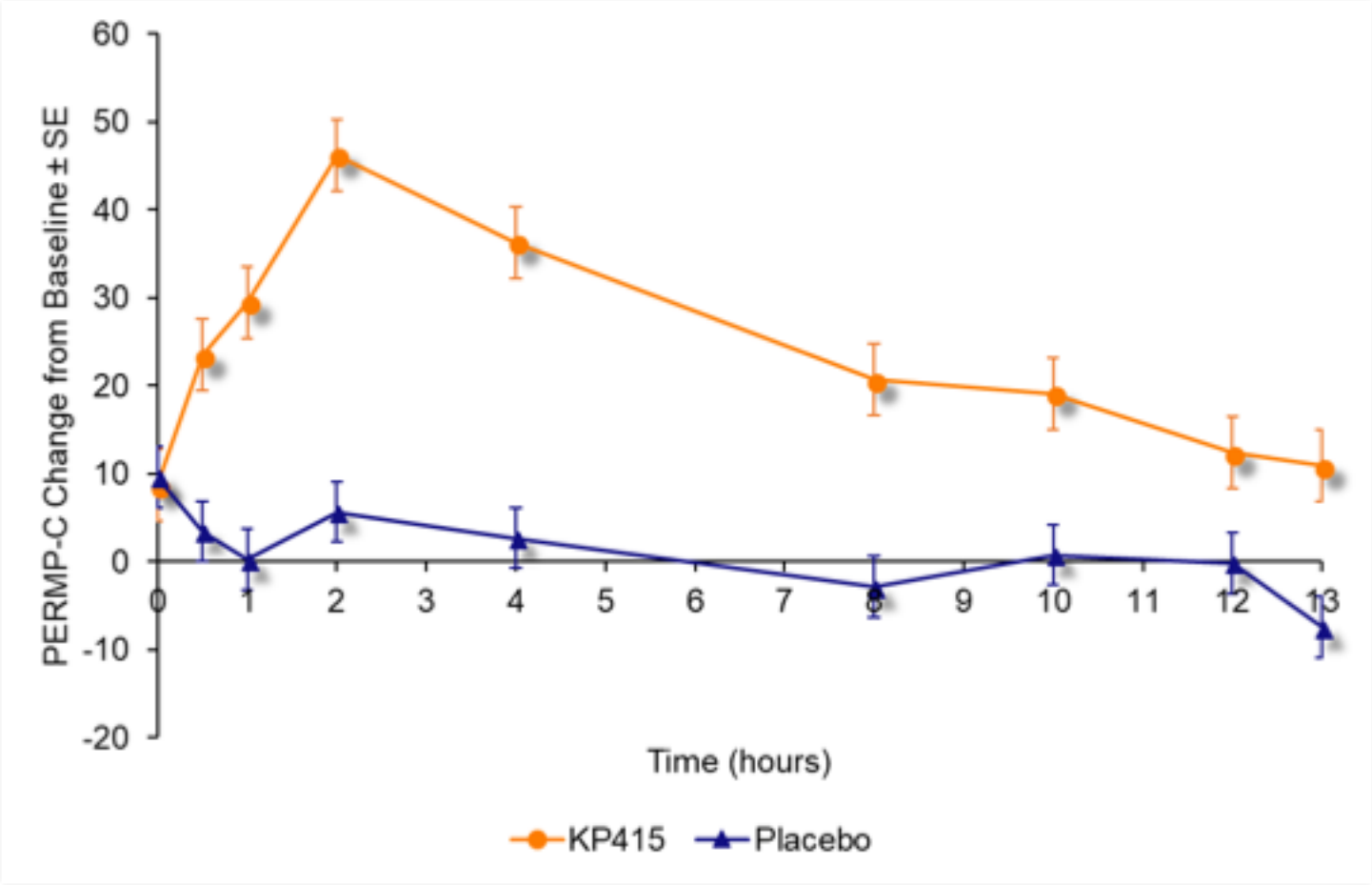
* Statistical model includes pre-dose Visit 5 or Visit 6 as covariate, respectively.

PERMP-A Change on Visit 6 Using Visit 5 Pre-Dose Baseline



LS means shown. Statistical model includes predose Visit 5 as covariate.
No meaningful difference between use of Visit 5 or Visit 6 baselines.

PERMP-C Change on Visit 6 Using Visit 5 Pre-Dose Baseline



LS means shown. Statistical model includes predose Visit 5 as covariate.
No meaningful difference between use of Visit 5 or Visit 6 baselines.

Summary and Next Steps

- KP415.E01 efficacy trial met its primary endpoint
- Trial results for onset and duration were confounded by pre-dose SKAMP-C baseline differences between Visit 5 and Visit 6 for patients randomized into the placebo arm of the trial
- Trial data using Visit 6 as the pre-dose baseline is supportive of a 30-minute onset and a 13-hour duration; secondary endpoints are similarly supportive
- Based on this and previously published trial data, we believe KP415 has an attractive safety and efficacy profile for the treatment of ADHD; NDA remains on track for submission in Q1 2019
- Strategic partnering discussions for KP415 continue based on:
  - Near-term ADHD prodrug commercial opportunity
  - KP415 IV HAP data, with more HAP data to come
  - Long patent life and potential NCE status

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