

Second Quarter 2017 Financial Results/Corporate Update

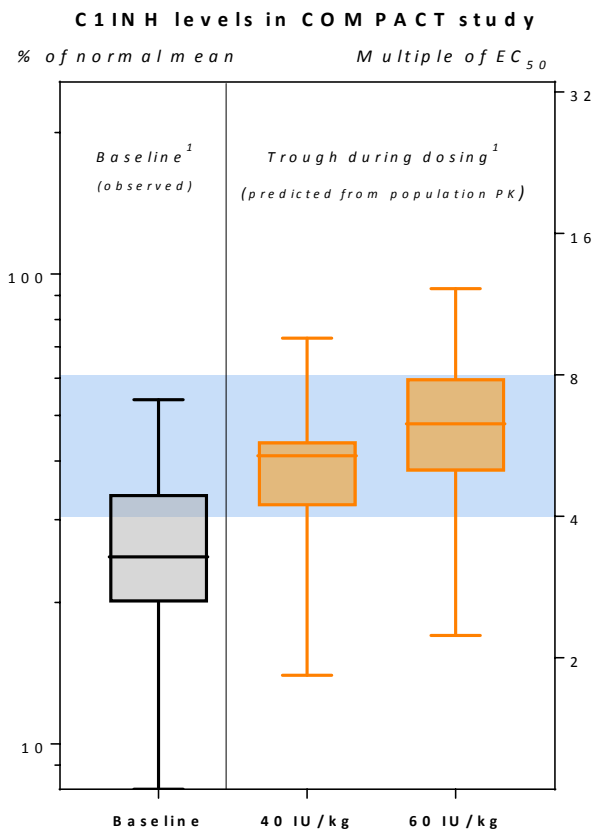
August 7, 2017

Forward-looking statement

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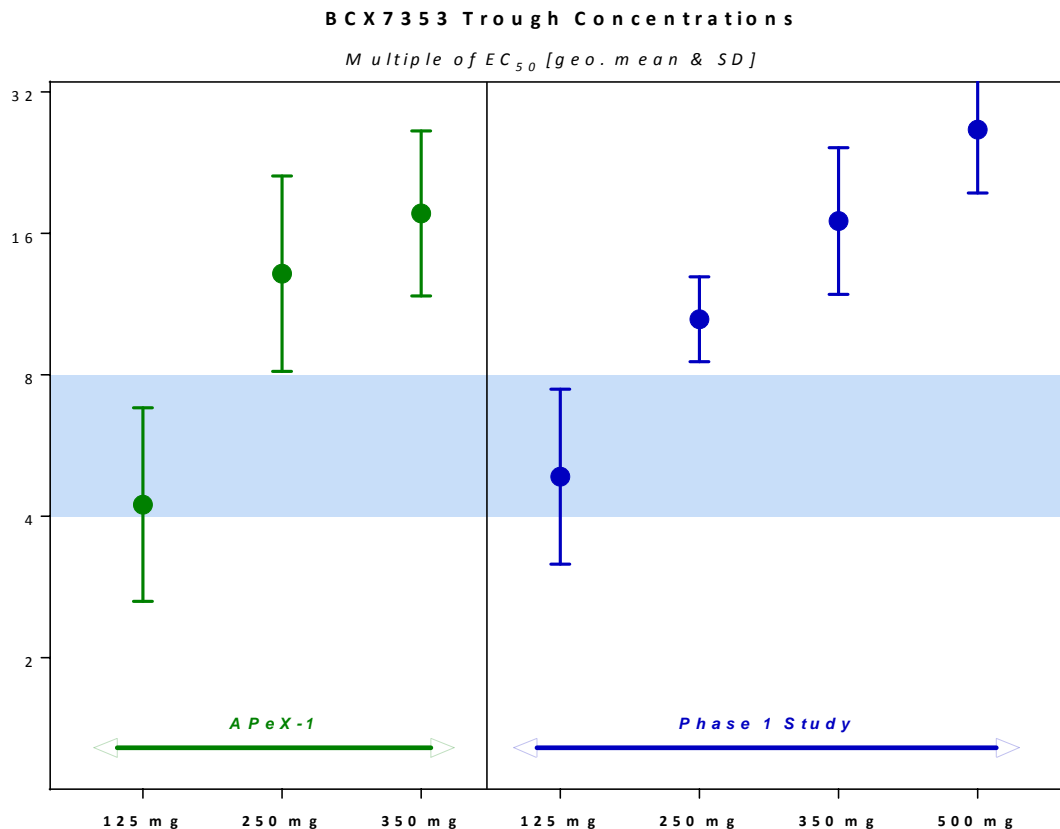
Exposure in APeX-1 and Phase 1 BCX7353 trials, and SC C1INH

CSL-830 Phase 3 study



C1INH levels at baseline and after SC dosing with CSL-830¹

BCX7353 APeX-1 & Phase 1

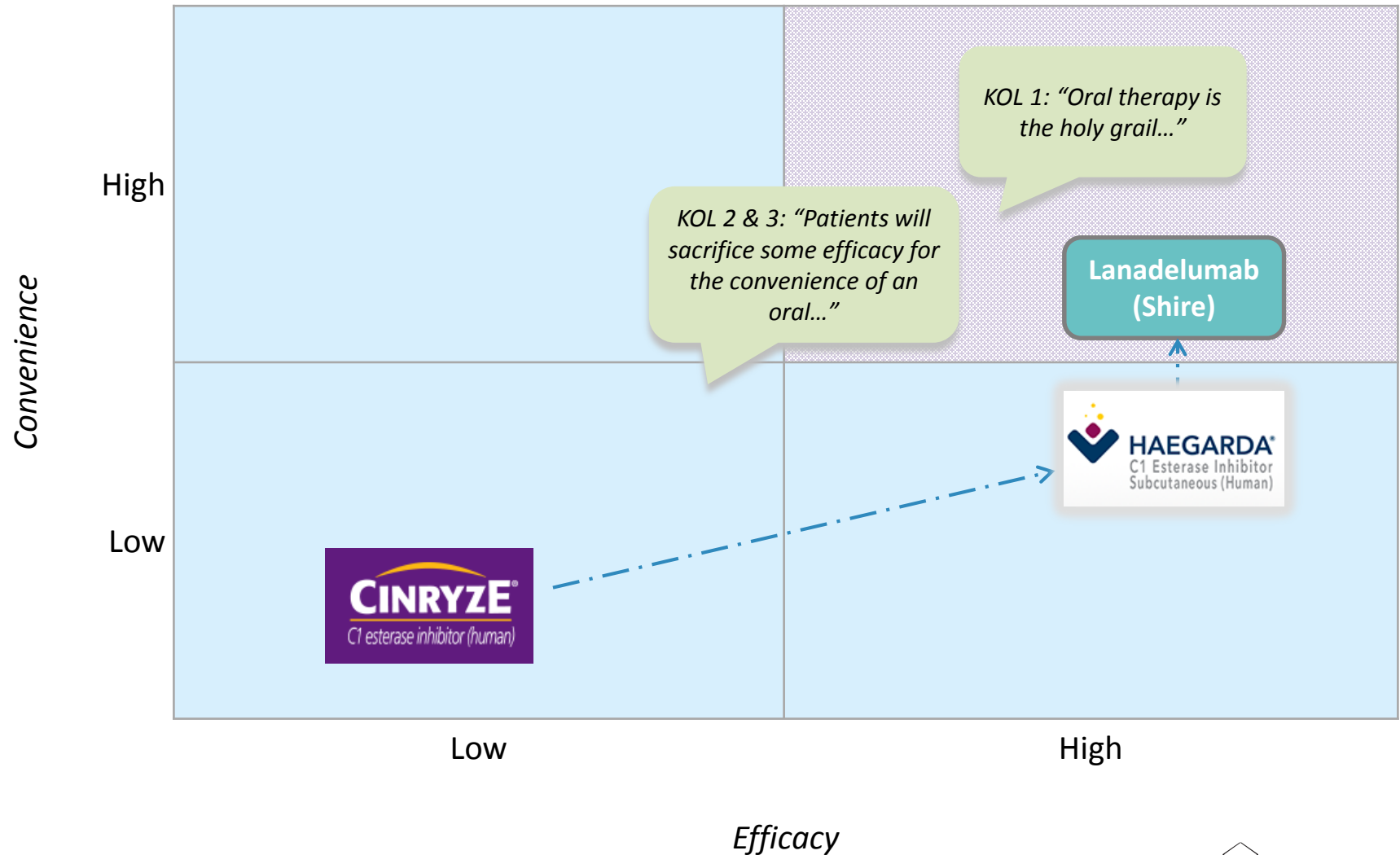


BCX7353 plasma concentrations at 24 hours post-dose

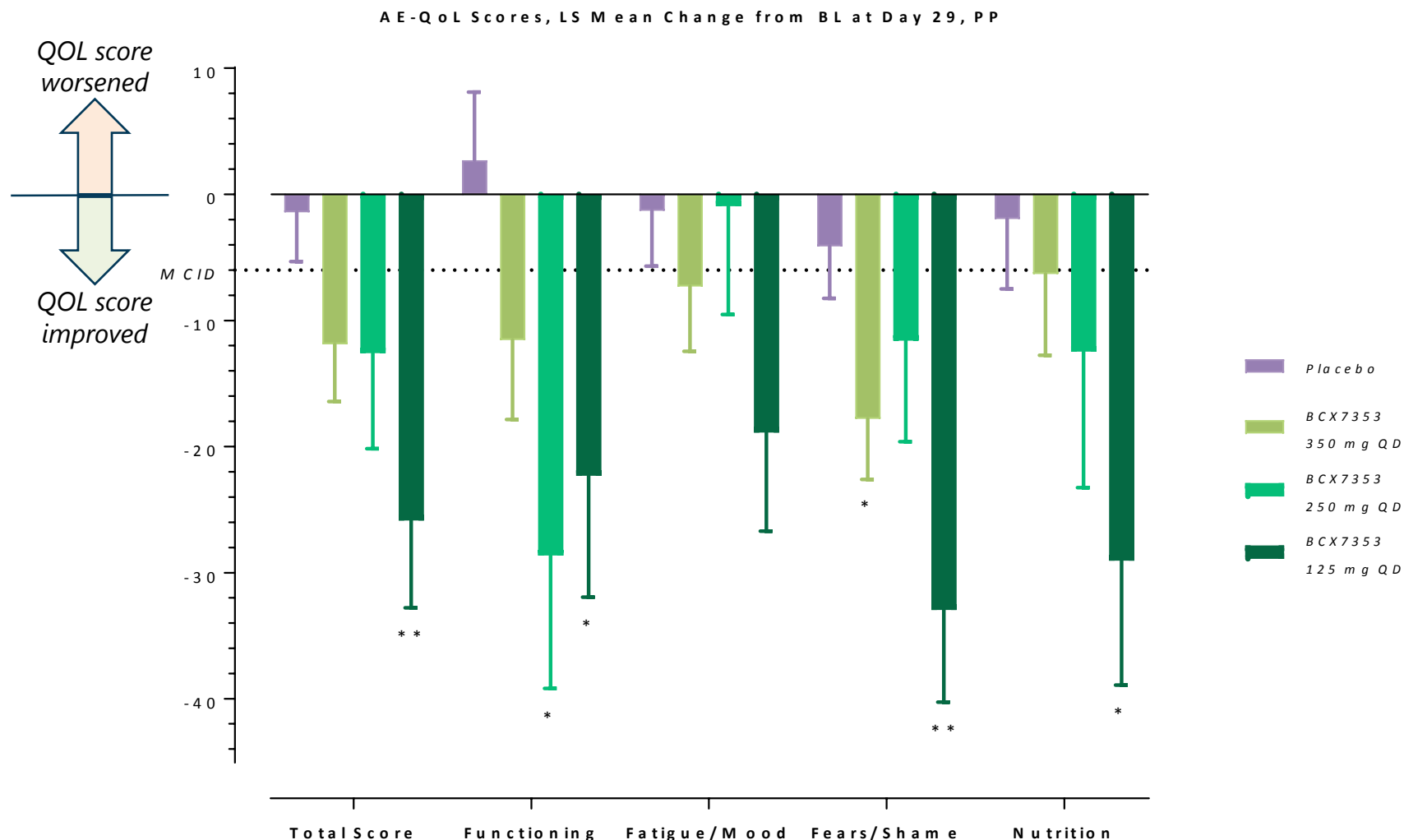
¹ Longhurst, H. et al. N Engl J Med 376, 1131-1140 (2017). Box plots represent median, 25th and 75th percentiles, minimum and maximum values. CSL-830 and BCX7353 data are from distinct clinical trials and no head to head study has been conducted.

Even as more prophylactic therapies become available for HAE, KOLs are still voicing a strong desire for an oral HAE therapy for their patients

HAE Prophylactic Products: *Positioning of Efficacy vs. Convenience*

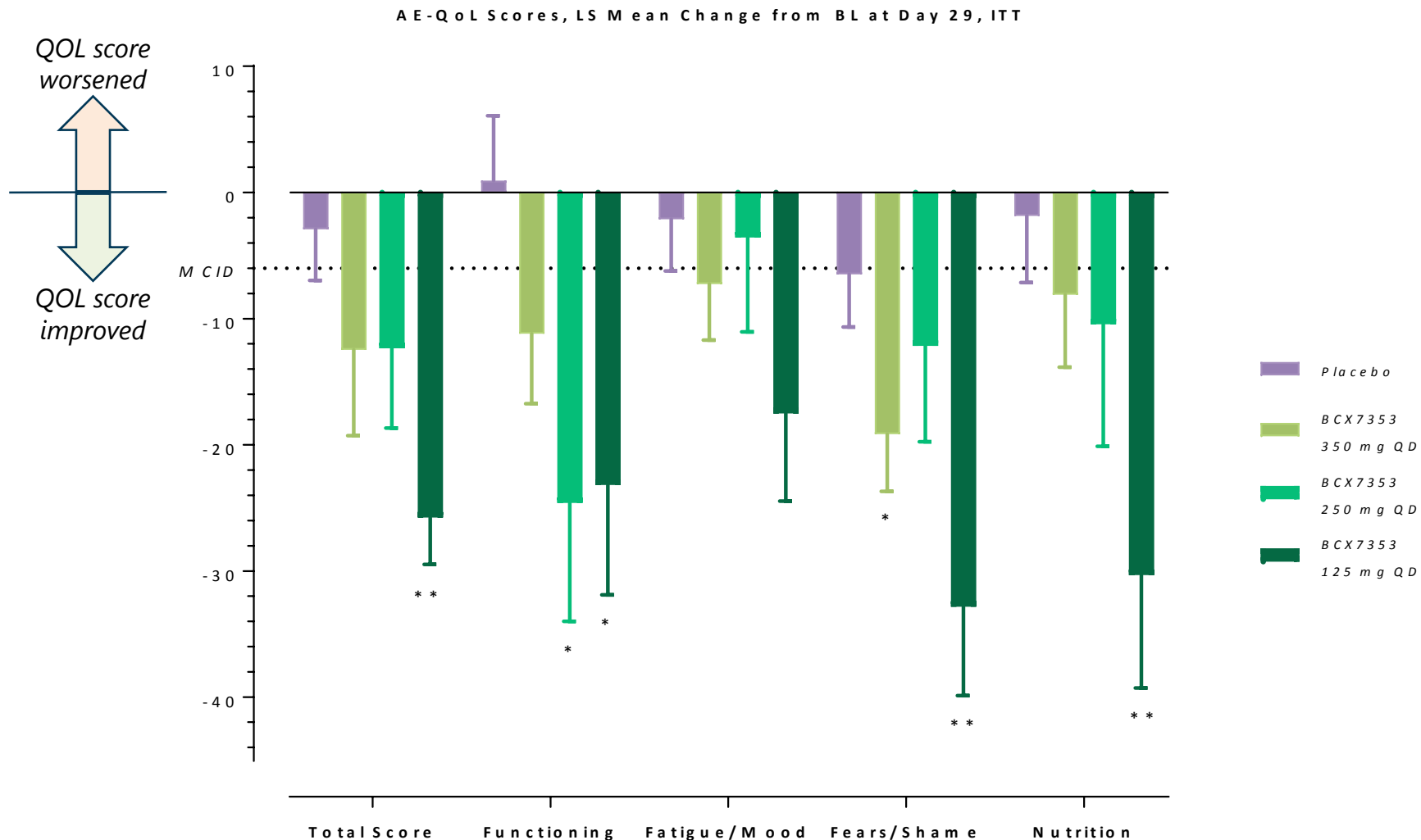


Quality of life scores, per protocol population analysis of change from baseline (Parts 1 & 2)



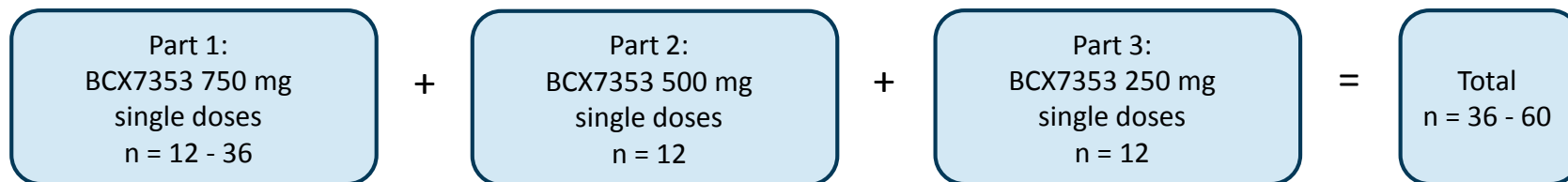
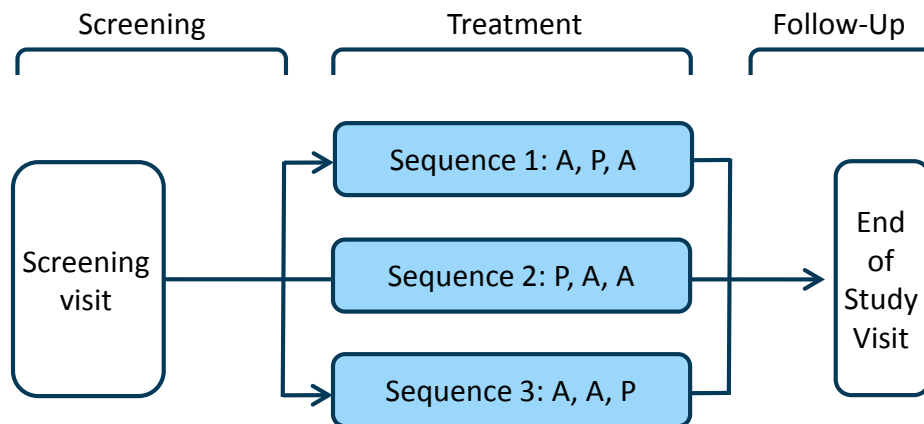
Difference in adjusted least square means are shown (Active treatment minus Placebo).
 ANCOVA Model includes terms of treatment and adjusted qualifying attack rate.
 Reductions (negative changes from BL) represent improvement in quality of life scores.
 MCID, minimum clinically important difference, -6 points (Weller, K. 2016. *Allergy* 71(8): 1203-1209.)
 * $p < 0.05$, ** $p < 0.01$, BCX7353 dose level compared with placebo

Quality of life scores, intent to treat analysis of change from baseline (Parts 1 & 2)



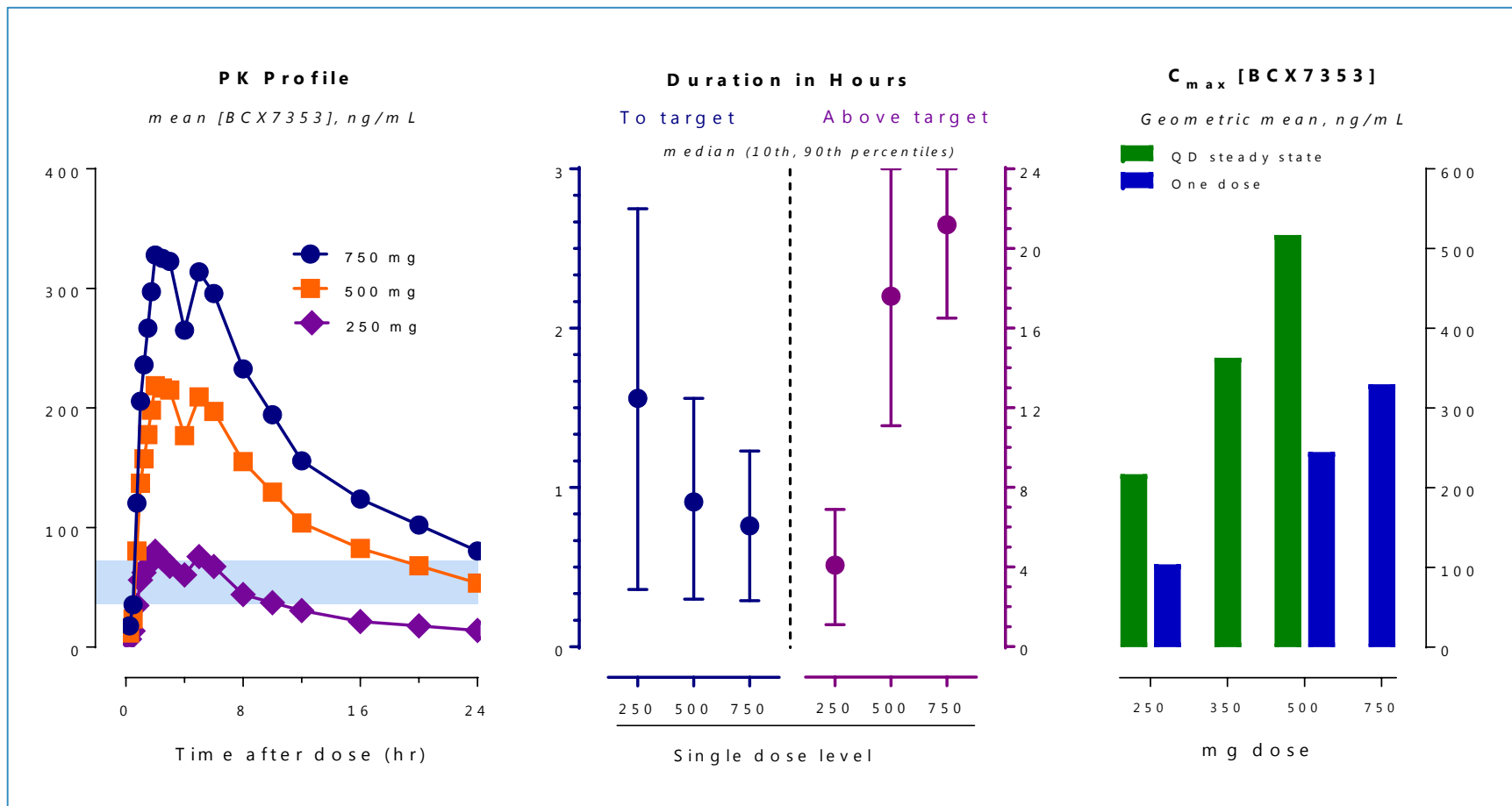
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ZENITH-1 trial design



- The ZENITH-1 trial will evaluate the potential for an oral liquid formulation of BCX7353 to treat acute angioedema attacks
- Each subject is intended to have 3 attacks treated with blinded study drug
 - 2 with BCX7353 (A) and 1 with Placebo (P)
- Subjects must have at least one attack per month for three months to qualify for the trial
- Primary efficacy endpoint: proportion of subjects with either improved or stable composite visual analog scale (VAS) score at 4 hours post-dose

ZENITH doses provide a range of target PK coverage



Simulations performed using PK data from phase 1 study of capsule solid dosage form of BCX7353.

C_{max} concentrations are actual for daily dosing steady state of 250 mg, 350 mg, and 500 mg in phase 1 study & single doses of 250 mg and 500 mg

C_{max} concentrations are simulated for 750 mg single dose

Blue shaded bar represents 4 to 8 x EC₅₀

Durations estimated for target of 8 x EC₅₀

Second quarter operating results

	Q2 2017	Q2 2016	Change Q2 2017 vs Q2 2016
<i>(in thousands, except per share amounts)</i>			
Revenues:			
Royalty revenue	\$ 489	\$ 629	(22%)
Collaborative and other R&D	2,610	4,158	(37%)
Total revenues	3,099	4,787	(35%)
Expenses:			
Research and development	15,759	14,166	11%
General and administrative	2,834	2,724	4%
Royalty	22	27	(19%)
Total operating expenses	18,615	16,917	10%
Loss from operations	(15,516)	(12,130)	28%
Interest and other income, net	203	147	38%
Interest expense	(2,094)	(1,421)	47%
Gain (loss) on foreign currency derivative	521	(2,877)	(118%)
Net loss	\$ (16,886)	\$ (16,281)	4%
Net loss per share - Basic & Diluted	\$ (0.21)	\$ (0.22)	(5%)
Net operating cash utilization	\$ 12,209	\$ 15,446	(21%)
Weighted average shares outstanding	80,418	73,695	

YTD operating results

	YTD 2017	YTD 2016	Change YTD 2017 vs YTD 2016
<i>(in thousands, except per share amounts)</i>			
Revenues:			
Royalty revenue	\$ 6,810	\$ 2,519	170%
Collaborative and other R&D	5,726	7,088	(19%)
Total revenues	12,536	9,607	30%
Expenses:			
Research and development	32,529	34,745	(6%)
General and administrative	5,892	5,936	(1%)
Royalty	316	104	204%
Total operating expenses	38,737	40,785	(5%)
Loss from operations	(26,201)	(31,178)	(16%)
Interest and other income, net	312	586	(47%)
Interest expense	(4,194)	(2,891)	45%
Loss on foreign currency derivative	(1,022)	(5,630)	(82%)
Net loss	\$ (31,105)	\$ (39,113)	(20%)
Net loss per share - Basic & Diluted	\$ (0.40)	\$ (0.53)	(25%)
Net operating cash utilization	\$ 21,048	\$ 37,891	(44%)
Weighted average shares outstanding	77,807	73,648	

Cash position & 2017 guidance (in millions)

Cash & investments at December 31, 2016	\$65
Cash & investments at June 30, 2017	\$96
Senior Credit Facility	\$23

Guidance for 2017:

Operating cash utilization	\$30 – 50
Operating expenses [#]	\$53 – 73

[#] Excludes equity-based compensation.