

Third Quarter 2016 Financial Results/Corporate Update

November 7th, 2016

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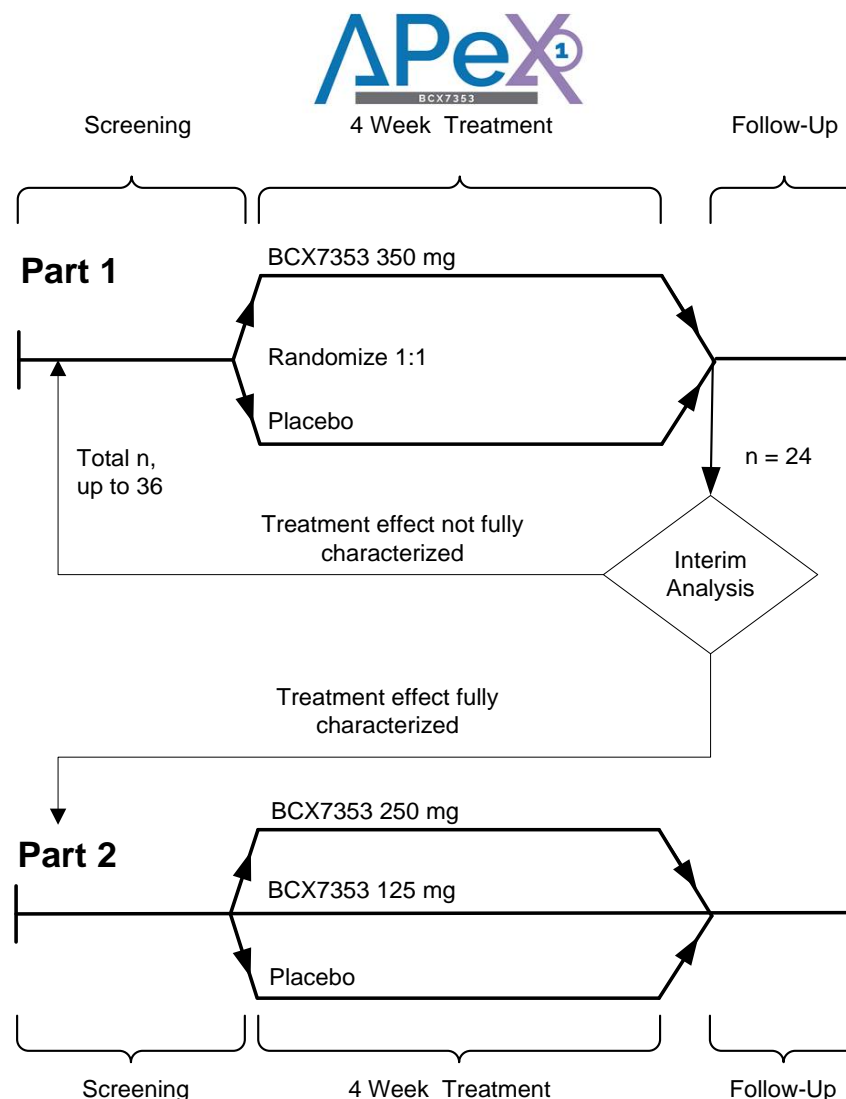
APeX-1: Phase 2 placebo-controlled trial of BCX7353 enrolling HAE patients

Design

- Part 1: proof of concept
 - 350 mg QD BCX7353 vs placebo
 - Interim analysis at n = 24
 - Option to add up to 12 subjects for total n = 36
 - Powered at 90% ($\alpha=0.05$) to detect a reduction in number of HAE attacks of $\geq 70\%$ on BCX7353
- Part 2: dose ranging
 - 250 mg QD and 125 mg QD BCX7353 and placebo
 - n = 14
 - 6:6:2 randomization

Endpoints

- Number of HAE attacks by treatment group will be analyzed as weekly attack rate, number of attacks, proportion of subjects with no attacks, number of attack-free days
- Additional endpoints include full safety assessments, QOL, PK/PD



Third quarter operating results

	Q3 2016	Q3 2015	Change Q3 2016 vs Q3 2015
<i>(in thousands, except per share amounts)</i>			
Revenues:			
Product sales, net	\$ -	\$ 5,699	(100%)
Royalty revenue	3,501	126	2679%
Collaborative and other R&D	4,262	5,162	(17%)
Total revenues	7,763	10,987	(29%)
Expenses:			
Cost of products sold	-	1,346	(100%)
Research and development	14,105	20,067	(30%)
General and administrative	2,756	2,731	1%
Royalty	143	5	2760%
Total operating expenses	17,004	24,149	(30%)
Loss from operations	(9,241)	(13,162)	(30%)
Interest and other income, net	109	134	(19%)
Interest expense	(1,465)	(1,241)	18%
Loss on foreign currency hedge	(931)	(352)	164%
Net loss	\$ (11,528)	\$ (14,621)	(21%)
Net loss per share - Basic & Diluted	\$ (0.16)	\$ (0.20)	(20%)
Net operating cash utilization	\$ 14,979	\$ 12,319	22%
Weighted average shares outstanding	73,734	73,262	

Nine month operating results

	9mo 2016	9mo 2015	Change 2016 vs 2015
<i>(in thousands, except per share amounts)</i>			
Revenues:			
Product sales, net	\$ -	\$ 6,236	(100%)
Royalty revenue	6,020	1,776	239%
Collaborative and other R&D	11,350	35,643	(68%)
Total revenues	17,370	43,655	(60%)
Expenses:			
Cost of products sold	-	1,361	(100%)
Research and development	48,850	53,711	(9%)
General and administrative	8,692	10,326	(16%)
Royalty	247	507	(51%)
Total operating expenses	57,789	65,905	(12%)
Loss from operations	(40,419)	(22,250)	82%
Interest and other income, net	695	367	89%
Interest expense	(4,356)	(3,862)	13%
(Loss) gain on foreign currency hedge	(6,561)	861	(862%)
Net loss	\$ (50,641)	\$ (24,884)	104%
Net loss per share - Basic & Diluted	\$ (0.69)	\$ (0.34)	103%
Net operating cash utilization	\$ 52,870	\$ 28,122	88%
Weighted average shares outstanding	73,677	72,752	

Cash position & 2016 guidance (in millions)

Cash & investments at December 31, 2015	\$100.9
Operating cash utilization through September 30, 2016	\$52.9
Cash & investments at September 30, 2016	\$68.7
Senior Credit Facility	\$22.7
Operating cash utilization	\$55 – 75
Operating expenses [#]	\$68 – 80
Cash runway	Early 2018

[#] Excludes equity-based compensation, and represents a modification from the previous range of \$78 - 98 million