First Quarter 2019 Results Call Corporate Update & Financial Results

May 8, 2019



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Agenda

• Corporate Update:

- APeX-2 24-week data readout on-track for Q2 2019
- Phase 3 ZENITH-2 study for oral BCX7353 for acute HAE to commence in summer 2019
- Phase 1 data with oral BCX9930 for complement-mediated diseases expected Q4 2019
 Jon Stonehouse President, Chief Executive Officer

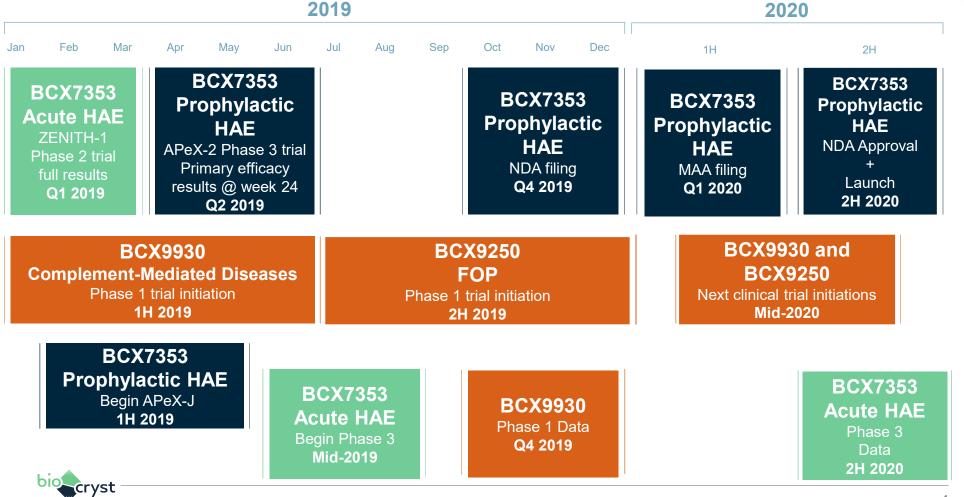
Clinical Update:

- APeX-2: 24-week data readout statistical analysis plan
 Dr. Bill Sheridan Chief Medical Officer
- Financial Update:

Thomas Staab – Chief Financial Officer

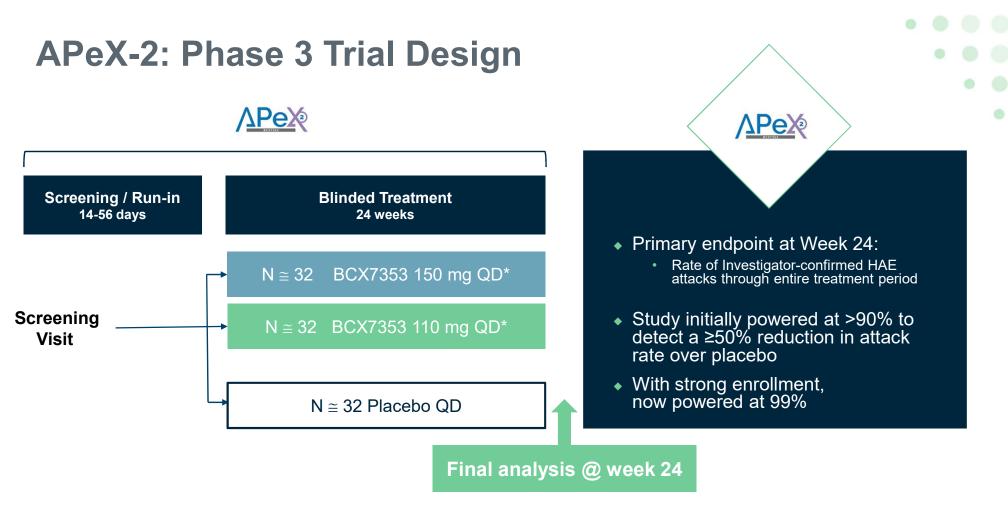
Summary and Q&A

Many Anticipated Milestones in 2019 - 2020



Clinical Update:

APeX-2: 24-week data readout statistical analysis plan



*Doses in Phase 2 APeX-1 were shown as the dihydrochloride salt: 150 mg = 175 mg dihydrochloride salt; 110 mg = 125 mg dihydrochloride salt



Financial Update:

Q1 2019

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First quarter operating results

(in thousands, except per share amounts)	Q1 2019	Q1 2018	Change Q1 2019 vs Q1 2018
Revenues:			
Product sales	\$ 1,679	\$ -	
Royalty revenue	2,322	3,661	(37%)
Collaborative and other R&D	1,886	315	499%
Total revenues	5,887	3,976	48%
Expenses:			
Cost of product sales	1,399	-	
Research and development	27,493	18,441	49%
General and administrative	6,238	7,609	(18%)
Royalty	87	140	(38%)
Total operating expenses	35,217	26,190	35%
Loss from operations	(29,330)	(22,214)	32%
Interest and other income, net	596	462	29%
Interest expense	(2,726)	(2,221)	23%
Gain (loss) on foreign currency derivative	406	(1,804)	(123%)
Net loss	\$ (31,054)	\$ (25,777)	21%
Net loss per share - basic & diluted	\$ (0.28)	\$ (0.26)	8%
Net operating cash utilization	\$ 27,108	\$ 22,901	18%
Weighted average shares outstanding	110,167	98,592	



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Cash position & 2019 guidance (in millions)

Cash & investments at December 31, 2018	\$128		
Cash & investments at March 31, 2019	\$122		
Senior Credit Facility ^A	\$50		
FY 2019 GUIDANCE			
Operating cash utilization	\$105 – 130		
Operating expenses ^B	\$120 — 145		

A - Credit Facility was modified in February 2019 to provide an additional \$20 upon closing and the ability to draw an additional \$50 of milestone-based tranches.

B - Excludes equity-based compensation.



Thank You... Questions and Answers

