

BioCRYST
PHARMACEUTICALS, INC.

Third Quarter 2017 Earnings Call Corporate Update & Financial Results

November 7, 2017

Agenda

- Introduction

Jon Stonehouse - President, Chief Executive Officer

- Clinical Update: Pathway to Approval Agreed

William Sheridan, MB BS - Senior Vice President, Chief Medical Officer

- HAE: Significant Market Opportunity

Lynne Powell - Senior Vice President & Chief Commercial Officer

- Financial Update: Strong Balance Sheet to Achieve Success

Thomas Staab - Senior Vice President, Chief Financial Officer

- Summary and Q&A

Forward-looking statements

BioCryst's presentation may contain forward-looking statements, including statements regarding future results, unaudited and forward-looking financial information and company performance or achievements. These statements are subject to known and unknown risks and uncertainties which may cause our actual results, performance or achievements to be materially different from any future results or performances expressed or implied in this presentation. You should not place undue reliance on the forward-looking statements. For additional information, including important risk factors, please refer to BioCryst's documents filed with the SEC, including its Annual Report on Form 10-K and subsequent Quarterly Reports on Form 10-Q, and located at <http://investor.shareholder.com/biocryst/sec.cfm>

Path forward is clear and opportunity is significant

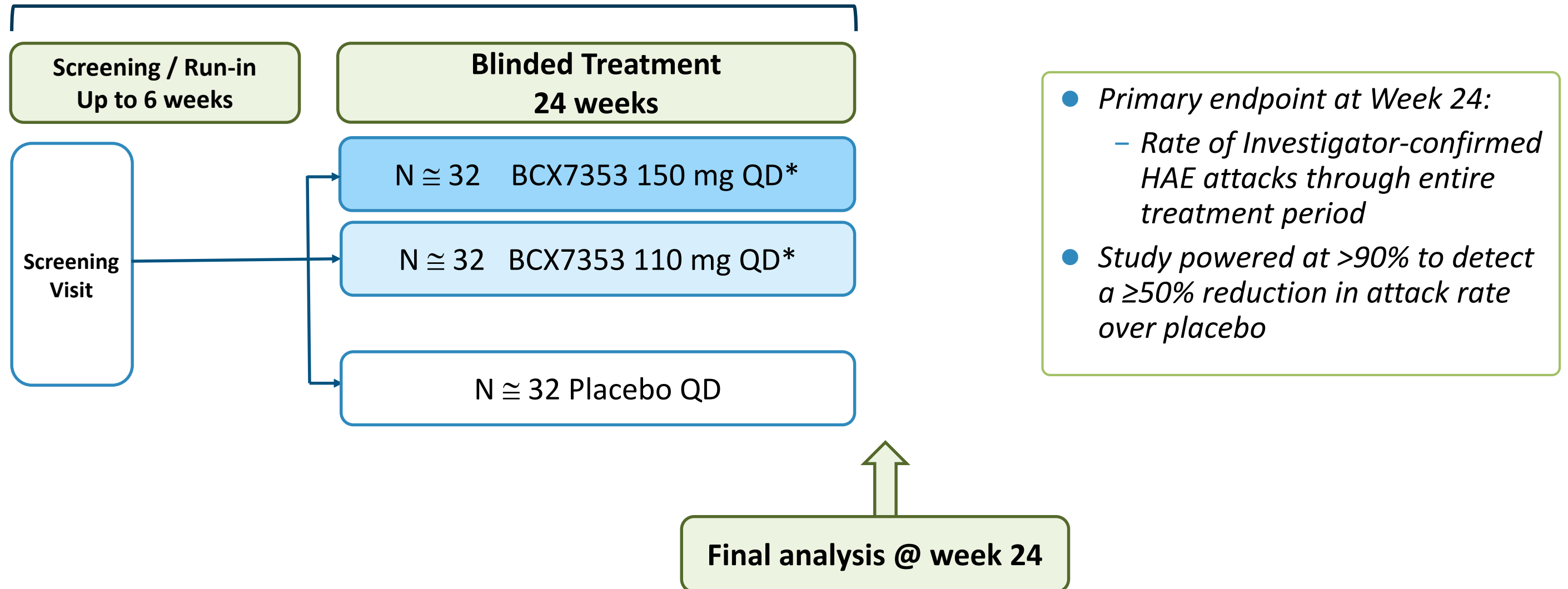
Agreed Path to Regulatory Filings

- **Regulatory interactions complete** – agreed on one pivotal and one long term safety trial
- **Designed to replicate APeX-1 success**
- **Start in 1Q18, finish APeX-2 in 1H19, file US NDA in 2H19**
- **U.S. Orphan Drug designation received**

Significant Market for Once-Daily Oral BCX7353

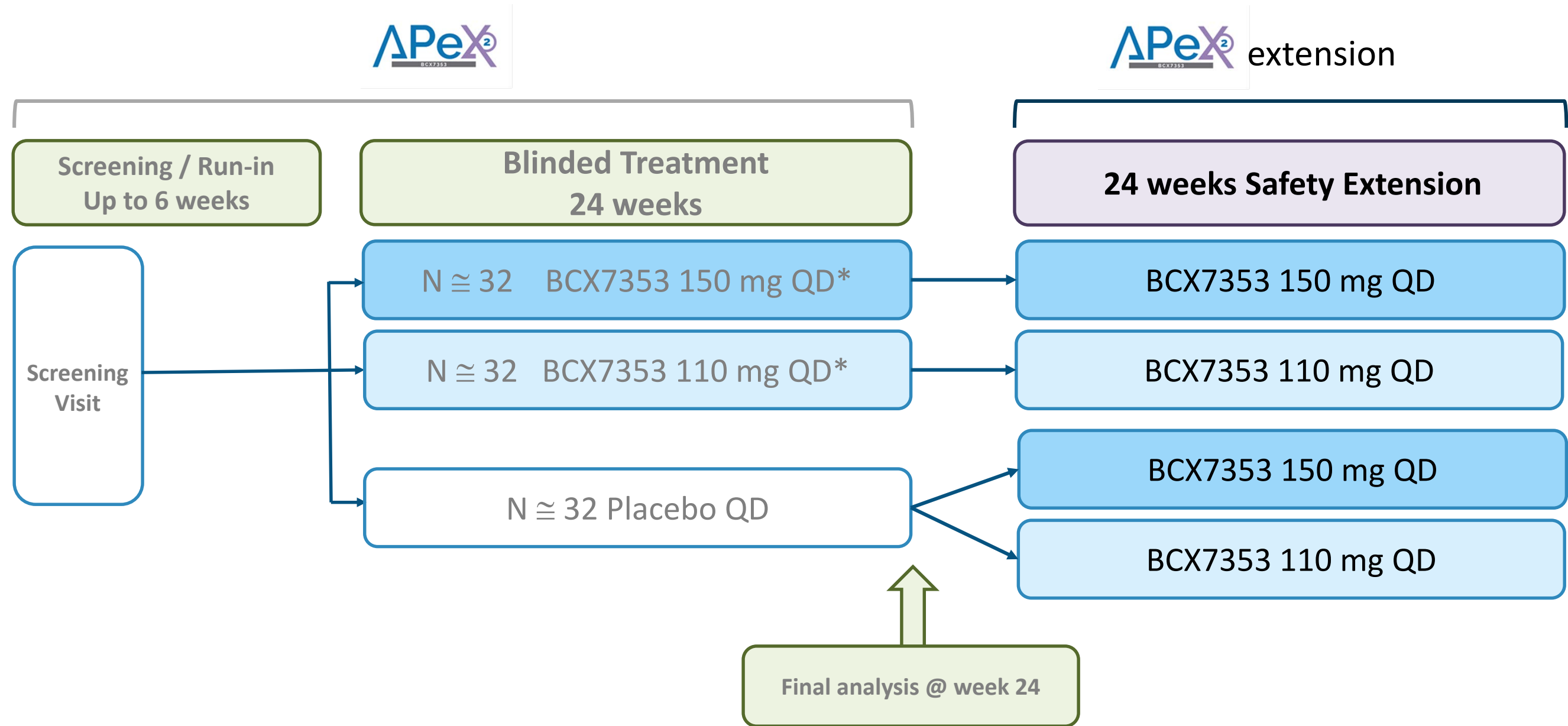
- **U.S. HAE market can be much larger** - currently \$1.5B, but only half of the ~6,500 patients are receiving C1INH prophylactic therapies
- **Market is evolving** - what is most important to patients and physicians is ease of administration
- **Once daily oral BCX7353 opportunity is significant** – we will get patients to switch from current therapy and the prophylactic market will grow

APeX-2 phase 3 trial design



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

APeX-2 phase 3 trial design – safety extension



***Doses in Phase 2 APeX-1 were shown as the dihydrochloride salt:
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APeX-S long-term safety study design



48 weeks treatment

$N \cong 80$ BCX7353 150 mg QD

$N \cong 80$ BCX7353 110 mg QD

Analyses as needed for
regulatory submissions

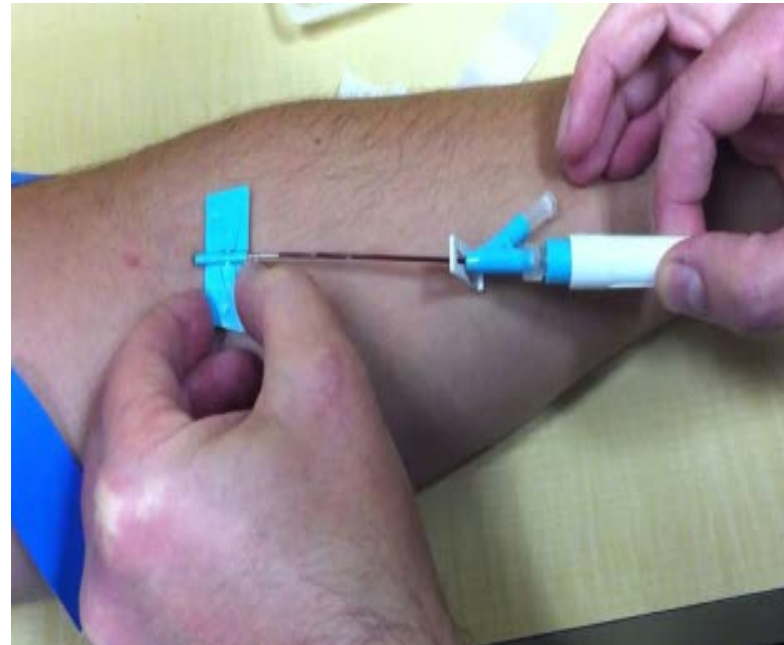
- **Endpoints:**
 - Long term safety of BCX7353
 - Durability of response
 - Quality of Life
- $N =$ approximately 200 subjects through 12 months in total from
 - APeX-2 safety extension
 - APeX-S
- APeX-1 subjects eligible

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Completing the revolution in care for HAE patients



Pre-2008
“The Dark Years”
*30% mortality **



2008-2016
“The IV Era”
Improved outcomes

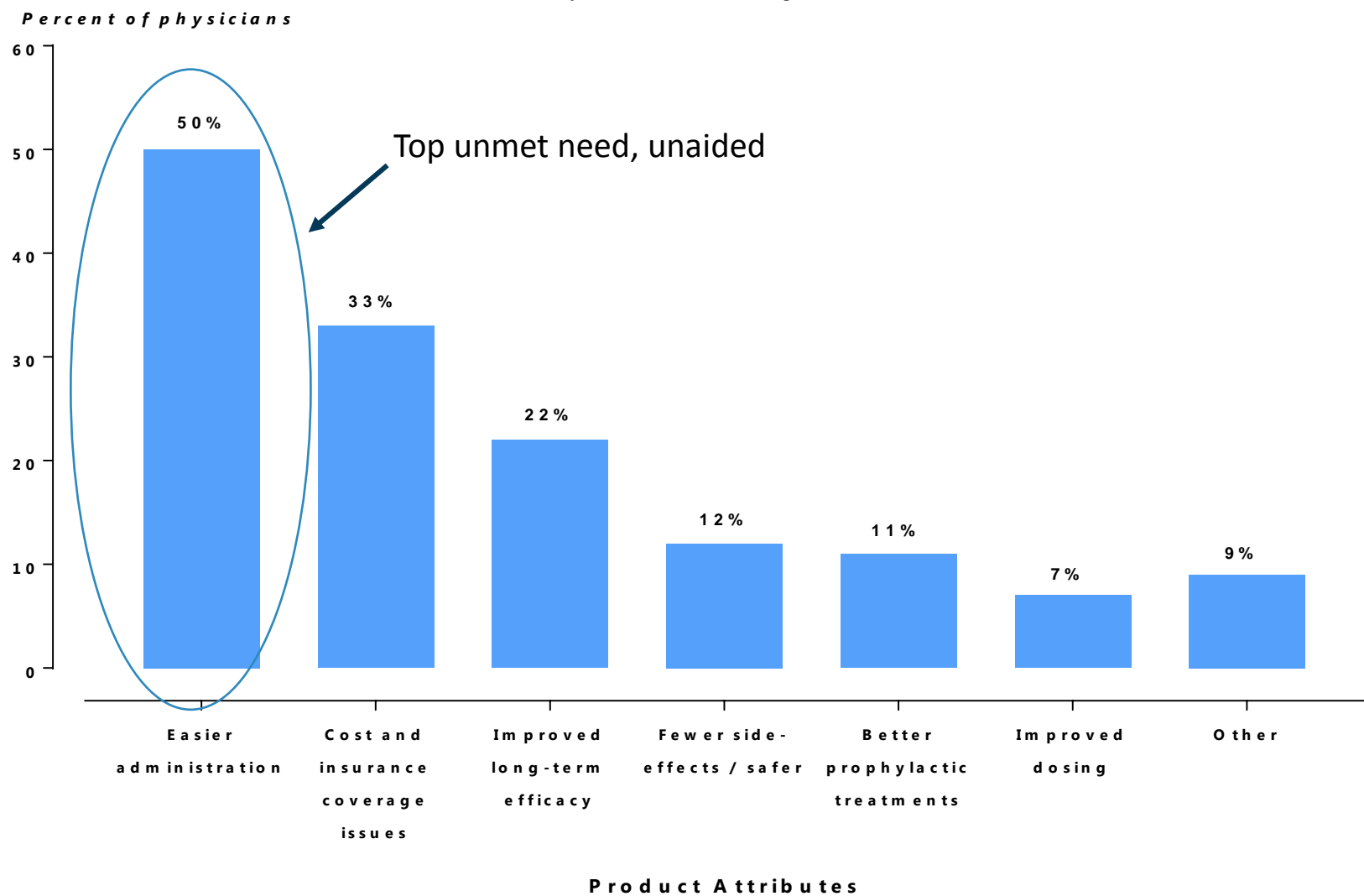


2017-2020
“Completing the Revolution”
High convenience/efficacy

* Source: Frank MM, Gelfand JA, Atkinson JP, Ann Intern Med. 1976;84(5):580.

Physicians and patients agree ease of administration is a high unmet need that will drive treatment choice

Physician Unmet Needs in HAE Treatments
N = 178 Physicians Treating HAE Patients



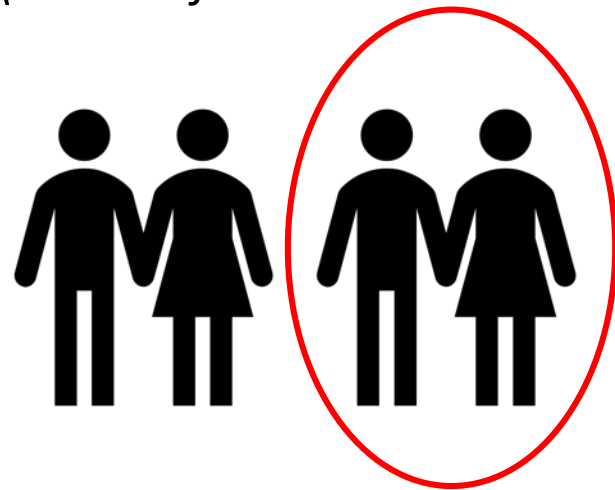
Public Meeting on Patient-Focused Drug Development for Hereditary Angioedema
September 25, 2017

HAE Patients note **'method of administration'** as most important factor driving treatment choice; over access/cost, dose, and side effect profile¹

Source: BCRX proprietary market research study, 3Q17; 1) RBC Capital Markets 'FDA Patient Event Highlights HAE Unmet Need, Challenges, Opportunities, 9/25/17

U.S. market is large with significant growth potential

~6,500 US patients
(Derived from US claims data)



50%
C1-Inhibitor
Prophylaxis



Source: BCRX proprietary market research for Prophy vs. Acute market split. Lexis-Nexis Risk Solutions- 'MarketView' Data (formerly HMS) claims data for ICD-9 & ICD-10 codes for HAE (August 2017, 12-month history). ICD10 code: D84.1 & ICD9: 277.6. Sales Data: BioCryst estimates based on Shire, CSL, Pharming public reports.

Third quarter operating results

	Q3 2017	Q3 2016	Change Q3 2017 vs Q3 2016
<i>(in thousands, except per share amounts)</i>			
Revenues:			
Product Sales	\$ 1,501	\$ -	100%
Royalty revenue	442	3,501	(87%)
Collaborative and other R&D	6,817	4,262	60%
Total revenues	8,760	7,763	13%
Expenses:			
Cost of products sold	1,142	-	100%
Research and development	17,509	14,105	24%
General and administrative	3,343	2,756	21%
Royalty	115	143	(20%)
Total operating expenses	22,109	17,004	30%
Loss from operations	(13,349)	(9,241)	45%
Interest and other income, net	225	109	106%
Interest expense	(2,140)	(1,465)	46%
Gain (loss) on foreign currency derivative	130	(931)	(114%)
Net loss	\$ (15,134)	\$ (11,528)	31%
Net loss per share - Basic & Diluted	\$ (0.18)	\$ (0.16)	13%
Net operating cash utilization	\$ 10,592	\$ 14,821	(29%)
Weighted average shares outstanding	83,570	73,734	

Cash position and 2017 guidance (in millions)

Cash & investments at December 31, 2016	\$65
Cash & investments at September 30, 2017	\$169
Senior Credit Facility	\$23

Guidance for 2017:

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

[#] Excludes equity-based compensation.

[@] We currently forecast our actual results to be in the upper-half of our 2017 Guidance.

Summary: path forward is clear and opportunity is significant

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Thank you... Questions and Answers
