Jefferies London Healthcare Conference

Tom Staab
Chief Financial Officer

Lynne Powell
Chief Commercial Officer

November 15, 2018

Forward Looking Statements

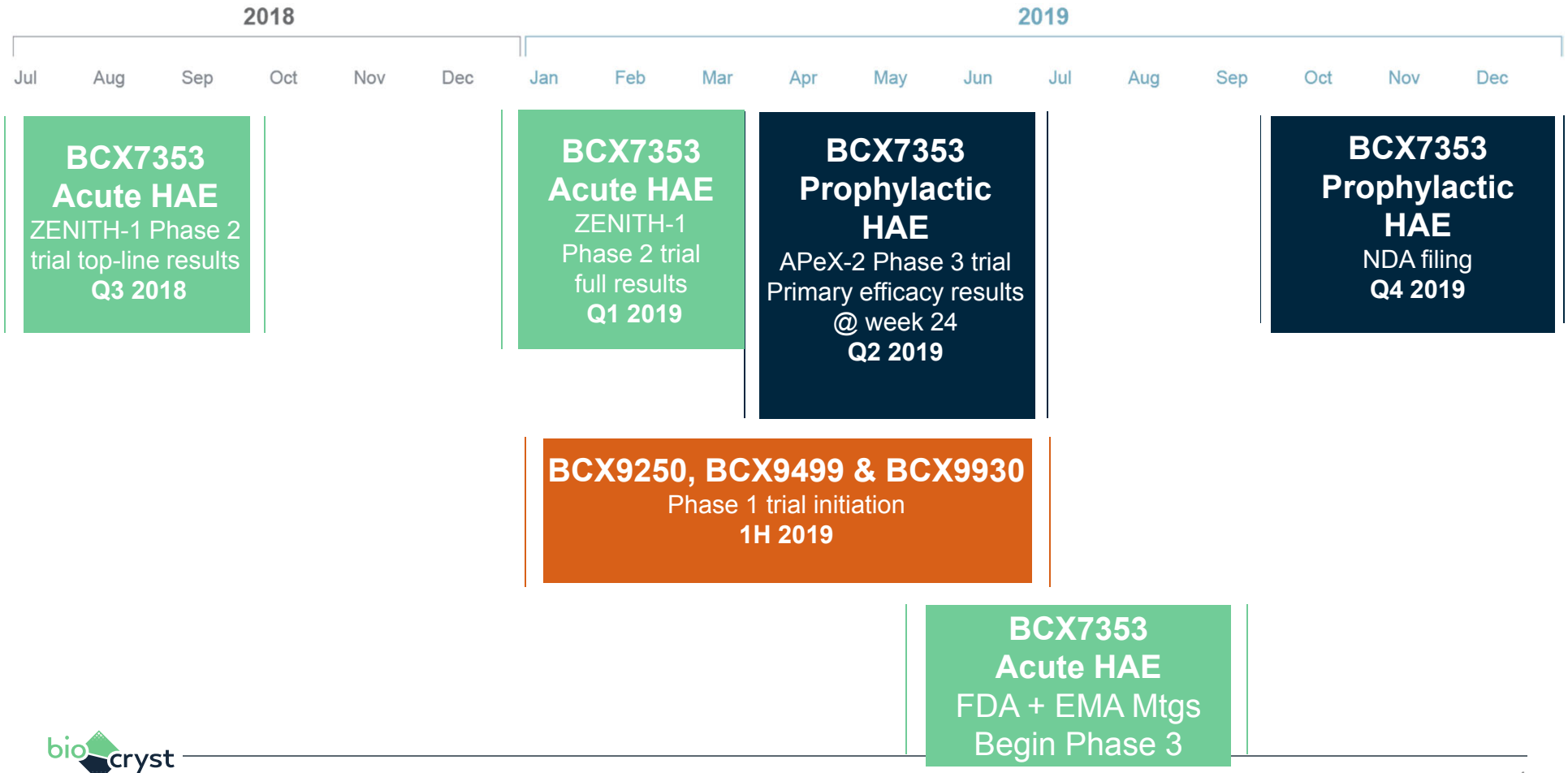
BioCryst's presentation may contain forward-looking statements, including statements regarding future results, performance or achievements. These statements involve known and unknown risks, uncertainties and other factors that may cause BioCryst's actual results, performance or achievements to be materially different from any future results, performance or achievements expressed or implied by the forward-looking statements. These statements reflect BioCryst's current views with respect to future events and are based on assumptions and are subject to risks and uncertainties. Given these uncertainties, you should not place undue reliance on these forward-looking statements. Some of the factors that could affect the forward-looking statements contained herein include: that the remaining cohorts of the ongoing ZENITH-1 trial may not be completed as expected; that the current results of the ZENITH-1 trial may not be predictive of future results, including the results of the remaining cohorts of ZENITH-1 and the APeX-2, APeX-S, and APeX-J trials; that developing BCX7353 for acute or prophylactic treatment may take longer or be more expensive than planned or may ultimately be unsuccessful; that producing a commercial formulation of BCX7353 may take longer than expected or may not occur as planned; that the Food and Drug Administration or other regulatory agencies may require additional studies beyond the studies currently planned, may not support trial designs, or may not provide regulatory clearances, which could result in delay of planned clinical trials; that we may never obtain market approval for BCX7353 or that commercialization of BCX7353 may ultimately be unsuccessful. Please refer to the documents BioCryst files periodically with the Securities and Exchange Commission, specifically BioCryst's most recent Annual Report on Form 10-K, Quarterly Reports on Form 10-Q, and Current Reports on Form 8-K, all of which identify important factors that could cause the actual results to differ materially from those contained in BioCryst's projections and forward-looking statements.

BioCryst's Robust Pipeline

	Lead Optimization	Pre-clinical	Phase 1	Phase 2	Phase 3	Filed	Approved
STRATEGY: Develop oral therapies for life-threatening, rare diseases							
BCX7353 – Oral Capsule (Prophylactic HAE)							
BCX7353 – Oral Formulation (Acute HAE)							
BCX9250 & BCX9499 (FOP)							
Other rare diseases							
SUPPORTING ASSETS: Externally funded, potential for capital infusions							
RAPIVAB® (peramivir injection)*							
Galidesivir (Broad spectrum antiviral) I.V.							

*Licensed to Seqirus, Shionogi and Green Cross

Multiple Anticipated Milestones



Cash Position & 2018 Guidance (in Millions)

Cash & investments at December 31, 2017	$159
Cash & investments at September 30, 2018	**$151**
Senior Credit Facility [A]	$30
FY 2018 GUIDANCE(stand-alone, as revised on July 11, 2018)	
Operating cash utilization	**$85 – 105**
Operating expenses [B]	**$90 – 110**

A - Credit Facility was enhanced in July 2018.

B - Excludes equity-based compensation.

Clinical Update:
APeX-2 Enrollment Complete
Data + NDA Timing On-track

Regulatory Agency Status for BCX7353

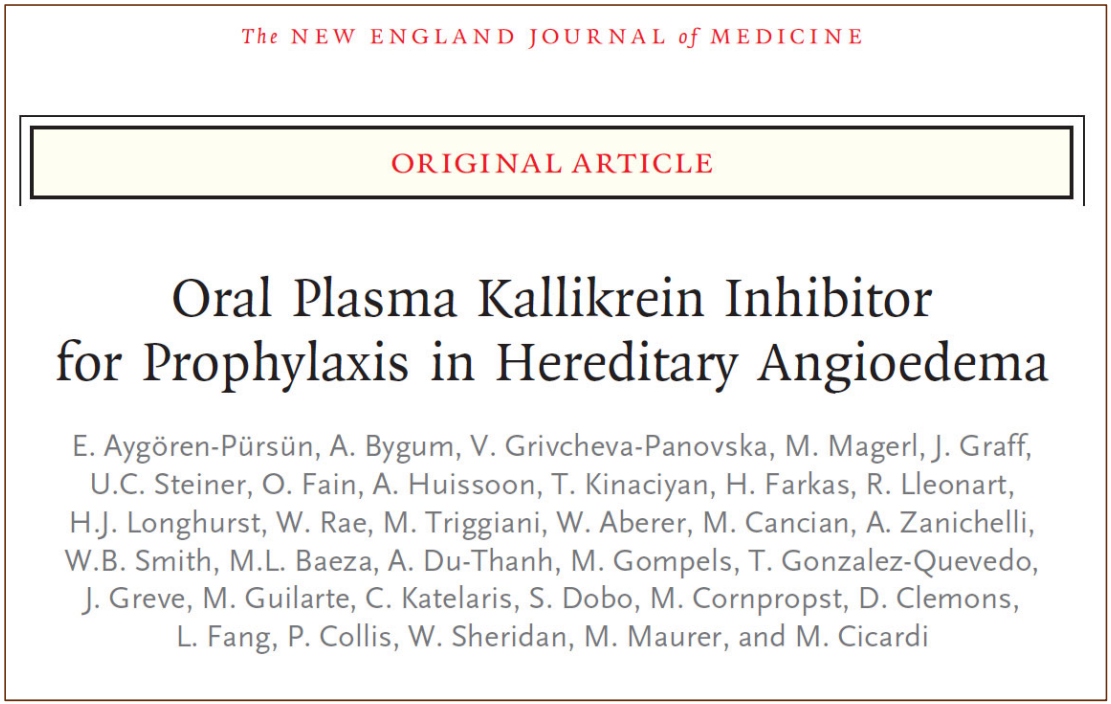
- Orphan Drug Designation 2017
- EOP2 2017
- Fast Track Designation

- Orphan Drug Designation 2018
- National Scientific Advice 2018
- Scientific Advice Process (EOP2 Equivalent) 2017

- UK Promising Innovative Medicine (PIM) 2018

- Formal Consultation Process (EOP2 equivalent) 2018
- Sakigake Designation 2015

BCX7353 phase 2 APeX-1 trial published, phase 3 trial well underway



The NEW ENGLAND JOURNAL of MEDICINE

ORIGINAL ARTICLE

Oral Plasma Kallikrein Inhibitor for Prophylaxis in Hereditary Angioedema

E. Aygören-Pürsün, A. Bygum, V. Grivcheva-Panovska, M. Magerl, J. Graff, U.C. Steiner, O. Fain, A. Huissoon, T. Kinaciyan, H. Farkas, R. Lleonart, H.J. Longhurst, W. Rae, M. Triggiani, W. Aberer, M. Cancian, A. Zanichelli, W.B. Smith, M.L. Baeza, A. Du-Thanh, M. Gompels, T. Gonzalez-Quevedo, J. Greve, M. Guilarte, C. Katelaris, S. Dobo, M. Cornpropst, D. Clemons, L. Fang, P. Collis, W. Sheridan, M. Maurer, and M. Cicardi

*Aygoren-Pursun, E. et al 2018 N Engl J Med **379**(4): 352-362*

APeX-2

Blinded Treatment
24 weeks

N ≅ 32 BCX7353 150 mg QD*

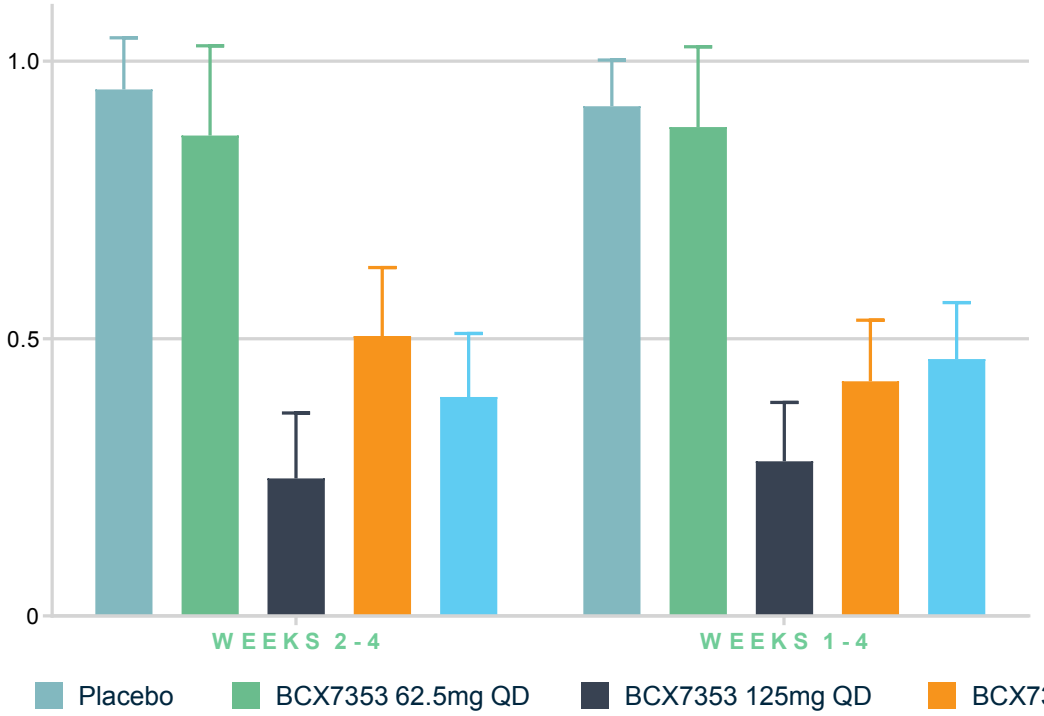
N ≅ 32 BCX7353 110 mg QD*

N ≅ 32 Placebo QD

Final analysis @ week 24

APeX-1: Overall Angioedema Attack Rate per Week, PP Population, Weeks 2-4 and 1-4

Attack Rate: LS Mean Attacks/Week



	62.5mg	125mg	250mg	350mg
WEEKS 2-4				
% Difference, Active-PBO	-9%	-74%	-47%	-58%
p-Value	0.657	<0.001	0.005	<0.001
WEEKS 1-4				
% Difference, Active-PBO	-4%	-70%	-54%	-50%
p-Value	0.818	<0.001	<0.001	<0.001

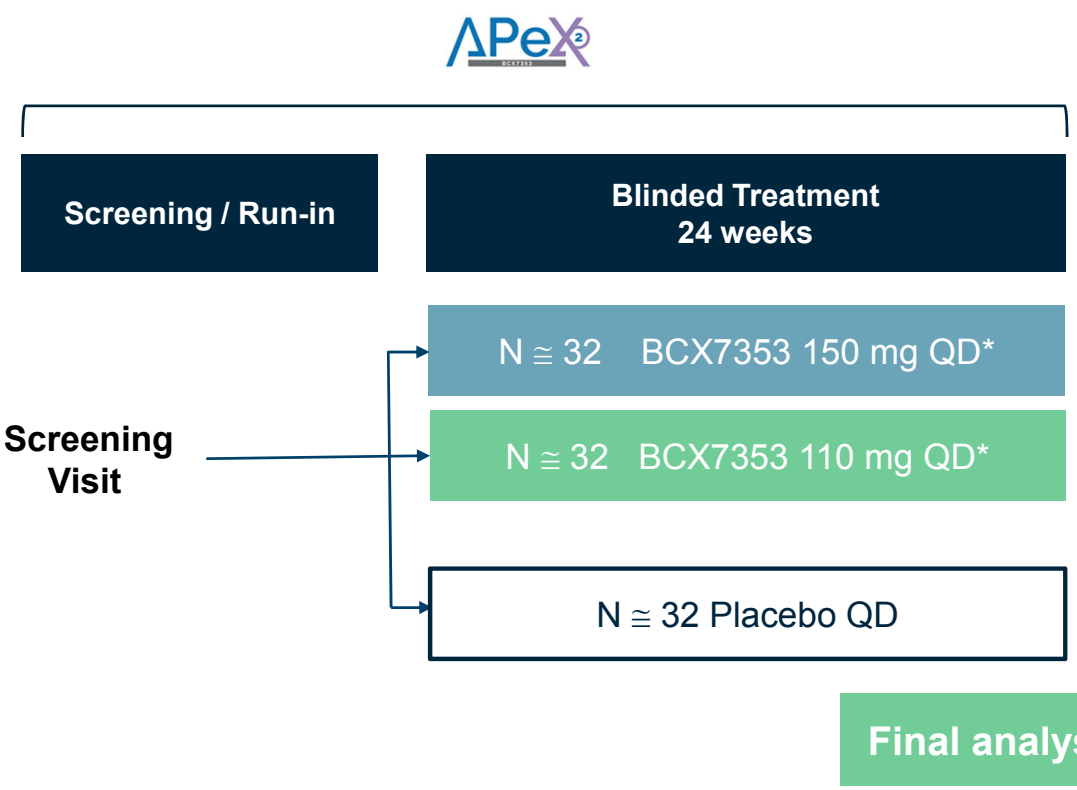
APeX-1: Treatment-Emergent Adverse Event Summary

Category	BCX7353 62.5 mg N = 7	BCX7353 125 mg N = 14	BCX7353 250 mg N = 14	BCX7353 350 mg N = 18	Placebo N = 22
Subjects with any TEAE[1], n (%)	4 (57)	7 (50)	11 (79)	14 (78)	15 (68)
Subjects with any Serious AE, n (%)	0	0	1 (7)[2]	0	0
Subjects with Drug-Related Grade 3 AE, n (%)	0	0	0	1 (6)	0
Subjects with AE Leading to D/C from Study Drug, n (%)	0	0	0	3 (17)	0
Non-drug-related, n (%)	0	0	0	1 (6)[3]	0
Drug-related, n (%)	0	0	0	2 (11)[4,5]	0

[1] TEAE- treatment-emergent adverse event.
[2] GI infection- investigator assessed as unrelated to study drug. Abdominal symptoms similar to several previous non-HAE-attack episodes occurring over past 3 years resulting in severe vomiting and diarrhea. Pt presented to ER and hospitalized for IV fluids and antiemetics as a precaution. No HAE acute attack meds given. Recovered and discharged the following day. Missed 1 day of dosing due to event.
[3] Pre-existing liver disorder (improved from baseline, but persisting). Previously reported in 1st interim analysis.
[4] n=1 Gastroenteritis with liver disorder (both assessed as related) (elevated ALT 1.9x ULN, GGT 5.4x ULN and ALP 1.6x ULN, with normal AST and bilirubin). Previously reported in 1st interim analysis.
[5] n=1 Vomiting/abdominal cramps. Previously reported in 2nd interim analysis.

Final analysis

APeX-2: Phase 3 Trial Design



- Primary endpoint at Week 24:
  - Rate of Investigator-confirmed HAE attacks through entire treatment period
- Study initially powered at >90% to detect a ≥50% reduction in attack rate over placebo
- With strong enrollment, now powered at 99%

Final analysis @ week 24

*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-S: Long-term Safety Study Design

48 Weeks Treatment

N ≅ 80 BCX7353 150 mg QD

N ≅ 80 BCX7353 110 mg QD

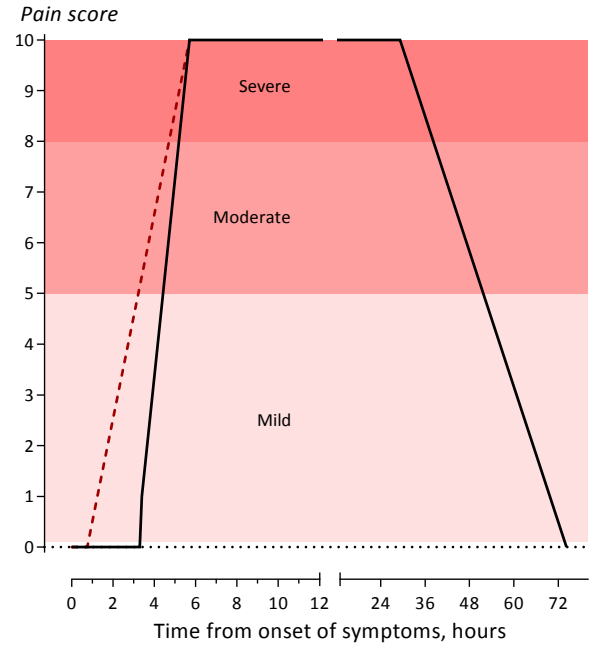
Analyses as needed for regulatory submissions

- Endpoints:
  - Long term safety of BCX7353
  - Durability of response
  - Quality of Life
- APeX-1 subjects eligible

- Safety database:
  - Up to 100 subjects at each dose level
  - Combination of APeX-2 extension and APeX-S

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

Angioedema Attacks in HAE Need Early Treatment to Prevent Severe Disability



[1] *Modified from Bork, K. 2006. Am J Gastroenterol* ***101****(3): 619-627*

Original Article

US Hereditary Angioedema Association Medical Advisory Board 2013 Recommendations for the Management of Hereditary Angioedema Due to C1 Inhibitor Deficiency

Bruce L. Zuraw, MD[a,b], Aleena Banerji, MD[c], Jonathan A. Bernstein, MD[d], Paula J. Busse, MD[e], Sandra C. Christiansen, MD[a,f], Mark Davis-Lorton, MD[g], Michael M. Frank, MD[h], Henry H. Li, MD[i], William R. Lumry, MD[j], and Marc Riedl, MD[k] *La Jolla, San Diego, and Los Angeles, Calif; Boston, Mass; Cincinnati, Ohio; New York and Mineola, NY; Durham, NC; Chevy Chase, Md; and Dallas, Tex*

"On-demand treatment of attacks is most effective when administered early in the attack. Patients should be counseled to treat as soon as the attack is clearly recognized." [2]

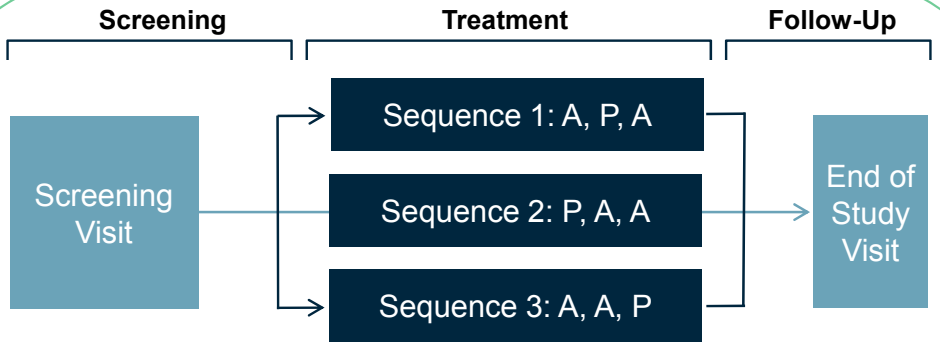
[2] *Zuraw, B. L. 2013 J Allergy Clin Immunol Pract* ***1****(5): 458-467*

ZENITH-1 is unique – designed to conform with current treatment paradigm of on-demand Rx

Drug *Study*	Cinryze *CHANGE*	Berinert *IMPACT-1*	Kalbitor *EDEMA-3*	Firazyr *FAST-3*	Ruconest *C-1310 Trial*	**BCX7353** ***ZENITH-1***
Years subjects enrolled	2005-2007	2005-2007	2005-2007	2009-2010	2011-2012	2017-2018
Route	IV infusion	IV infusion	SC injection	SC injection	IV infusion	PO (liquid)
Duration of symptoms prior to Rx	≤ 4 hours	≤ 5 hours	≤ 8 hours	6 to 12 hours	≤ 4 hours	≤ 1 hour
Location of treatment	Clinic	Clinic	Clinic	Clinic	Clinic	Home
Duration of observation by HCP	≥ 4 hours	≥ 4 hours	≥ 4 hours	≥ 8 hours	6 hours	none
Treatment administration	HCP	HCP	HCP	HCP	HCP	Patient
Availability of self-administered rescue Rx	None	None	None	None	None	icatibant pdC1INH rhC1INH
Availability of HCP-administered rescue Rx	Second dose of blinded study drug	Second dose of blinded study drug	Opiates, antiemetics	icatibant pdC1NH	rhC1INH icatibant pdC1NH ecallantide	icatibant pdC1INH rhC1INH

ZENITH-1 Phase 2 Placebo-controlled Trial of At-home Self-administered Oral Treatment

Trial methods are aligned with the current guidelines for on-demand treatment[1,2]



A- Active BCX7353; P- Placebo

Trial part	Dose level	N of subjects
Part 1	750 mg	36
Part 2	500 mg	12
Part 3	250 mg	12
Total		*60*

Subjects in Part 1	N
Subjects randomized	36
Subjects treated	33
Completed 3 on-study attacks	30

Attacks in Part 1	BCX7353	Placebo
Attacks treated, N	64	31
Time from onset of symptoms to study drug administration, min *median (range)*	35 (10-90)	35 (10-90)
Baseline mean VAS score	14.0	15.0

BCX7353 Showed Clinically Meaningful and Statistically Significant Benefits

Efficacy Endpoint	BCX7353	Placebo	Difference	P value
VAS Endpoints				
Change from baseline in composite VAS score through 4hr (Least Squares Mean)	-3.9	+3.1	-6.98	0.0024
Proportion of attacks with improved or stable composite VAS score through 4hr	67.7%	46.7%	+21.0%	0.0387
Proportion of attacks with improved or stable composite VAS score through 24hr	62.5%	35.5%	+27.0%	0.0125
Time to stable or improved composite VAS (median)	1 hr	2 hr	-1 hr	0.0452
Time to ≥ 50% reduction in composite VAS through 24 hr (median)	8hr	24hr	-16hr	0.0671
Time to almost complete symptom relief [all 3 individual VAS <10] (median)	23.1 hr	23.6 hr	-0.5 hr	0.6767
Standard of Care Treatment Endpoints				
Proportion of attacks requiring standard of care treatment through 24hr	29.7%	61.3%	-31.6%	0.0029
Time to standard of care acute attack treatment (median)	> 24 hr	14hr	> +10 hr	0.0043
Patient Global Assessment Endpoints				
Proportion of attacks with no or mild symptoms through 4 hr	69.4%	50.0%	+19.4%	0.0552
Proportion of attacks with no or mild symptoms through 24 hr	64.1%	32.3%	+31.8%	0.0038
Proportion of attacks with improved or stable symptoms through 24 hr	64.1%	35.5%	+28.6%	0.0092
Proportion of attacks with improved or stable symptoms through 4 hr	82.3%	60.0%	+22.3%	0.0192
Time to initial symptom relief (median)	5.1 hr	19.4 hr	-14.3 hr	0.0978
Time to complete symptom relief (median)	35.1 hr	41.3 hr	-6.2 hr	0.8900

BCX7353 Safe and Well Tolerated in ZENITH-1

Category	BCX7353	Placebo
Number of subjects	33	31
Number of attacks treated*	64	31
Number of attacks with a reported treatment-emergent adverse events (TEAE)	16 (25.0%)	7 (22.6%)
Number of attacks with a serious TEAEs	0	1 (3.2%)
Number of attacks with a drug-related TEAEs as assessed by investigator	7 (10.9%)	4 (12.9%)
Number of attacks with TEAEs leading to permanent discontinuation from study drug	1 (1.6%) ‡	1 (3.2%) §
Number of attacks with TEAEs of Grade 3 or Grade 4	0	0
Number of attacks with TE lab abnormalities of Grade 2, 3, or 4	0	0
Number of attacks with drug-related TEAEs of Grade 3 or 4	0	0
Number of attacks with drug-related serious TEAEs	0	0
Most common adverse events		
Nasopharyngitis	*4 (6.3%)*	*1 (3.2%)*
Diarrhea	*3 (4.7%)*	*0*
Headache	*3 (4.7%)*	*0*

** To account for observation bias, the reported rates take into account the proportion of time considered treatment emergent for BCX7353 and the proportion of time considered treatment emergent for placebo, by using the denominator of number of attacks treated.*

‡ Discontinuation on BCX7353 occurred in a subject who developed a small red macule on the forearm 11 hours after taking BCX7353 for an HAE attack occurring in the same anatomic location. The macule lasted for 4 hours and resolved without treatment.

§ Discontinuation on placebo occurred in a subject who experienced abdominal pain on both active and placebo drug. The decision to stop study drug occurred after the placebo dose.

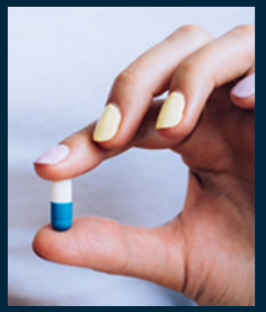
Commercial Update:
Patients Want an Oral Therapy

Lynne Powell
Chief Commercial Officer

BCX7353 - A New Approach to Hereditary Angioedema Treatment

ANNUAL TREATMENT

ONCE A DAY



Unpredictable, debilitating, potentially life-threatening swelling attacks

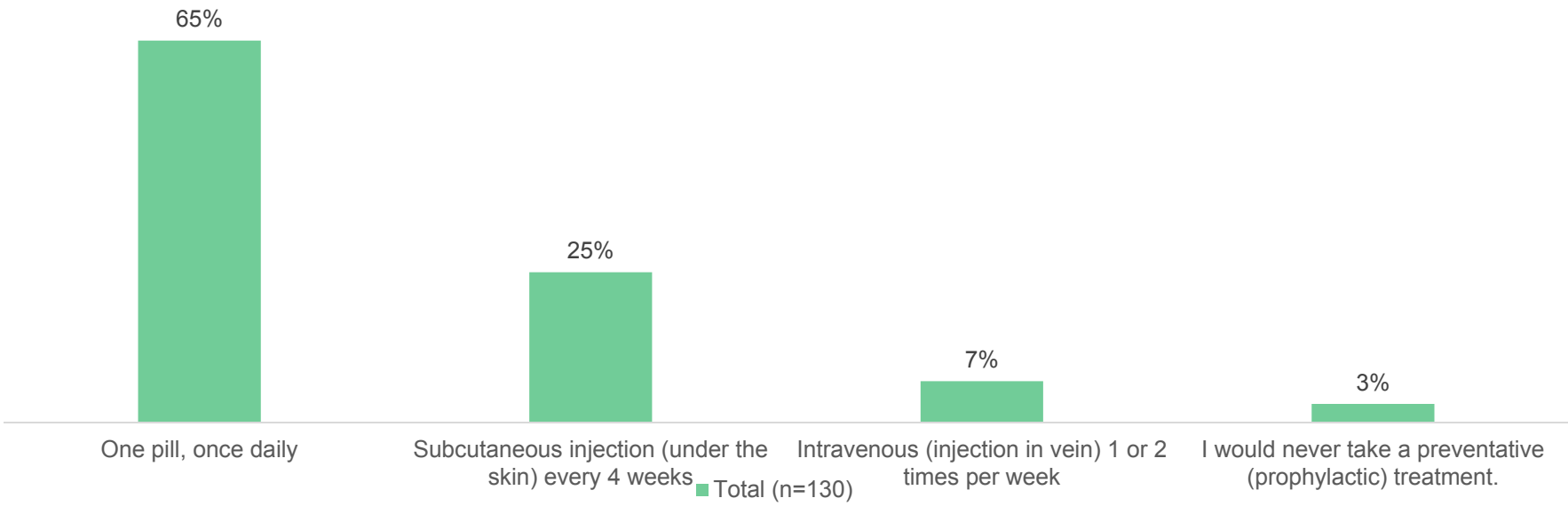
1 in 50,000 people affected worldwide

Injectables are current standard of care

BCX7353 is an oral once daily selective inhibitor of plasma kallikrein currently in Phase 3

HAE patients want oral prophylaxis

Two-thirds would prefer daily oral therapy



Source: BCRX market research; n=130 adult patients diagnosed with HAE in the US

Question: When thinking about taking a medication to prevent your HAE attacks (prophylactic treatment), which medication would you prefer assuming each is equally safe and effective and costs the same amount per year?

Patients and HCPs are attracted to BCX7353

A profile similar to APeX-1 results will draw strong interest

59% of patients

are very likely to ask their doctor about BCX7353

→ Of these patients

61%

Unaided, cited ease of use/lack of injection as top reason

64% of allergists

are very likely to prescribe BCX7353

→ Of these allergists

89%

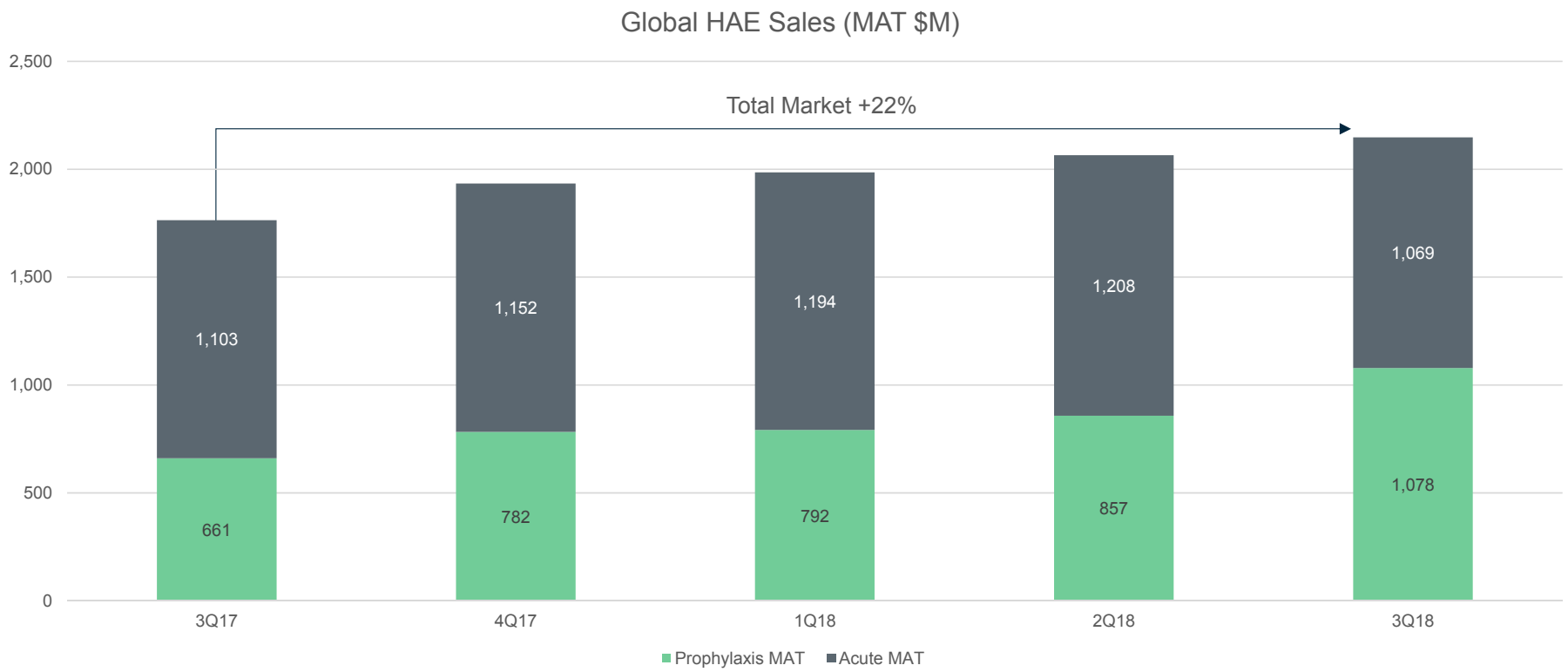
Unaided, cited ease of use/lack of injection as top reason

Source: BCRX market research; n=101 adult patients diagnosed with HAE in the US and n=101 Allergist/immunologists treating HAE patients in the US. BCX7353 profile tested with 75% attack reduction and possibility of mild gastrointestinal side effects.

Patient question: Please assume that Treatment X is FDA-approved and has comparable pricing and insurance access as available alternatives. How likely are you to ask your physician about Treatment X?
Allergist Question: Please assume that Treatment X is FDA-approved and has comparable pricing and insurance access as available alternatives. How likely are you to prescribe Treatment X to treat your HAE patients?
Very likely = 6 or 7 on 7-point scale

Annualized HAE Sales Over $2.1B Through 3Q18

New product launches driving prophylaxis past 50% of MAT sales



Sales based on actual reported sales for Shire products; actual reported sales for Pharming through 2Q19 and estimates through Q3; and estimates for CSL products based on publicly reported data and comments in 2017 and 2018.

MAT= Moving 12 Month Average

BioCryst Positioned for Success with Multiple Upcoming Data Milestones

- Building a company to develop novel oral therapies for rare diseases, which help patients experience a normal quality of life
- Starting with kallikrein inhibitors for HAE
  - BCX7353 for both prophylaxis and acute therapy
  - First oral therapy—a big deal for patients
  - Strong safety and efficacy profile in clinical trials
- Pipeline behind BCX7353—Approaching the clinic
  - FOP
  - Additional indication
- Well capitalized
- Next 18 months: ZENITH-1, APeX-2, APeX-S, BCX7353 NDA + MAA, New Phase 1 program

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