Third Quarter 2018 Results Call
Corporate Update & Financial Results

November 6, 2018

Forward-Looking Statements

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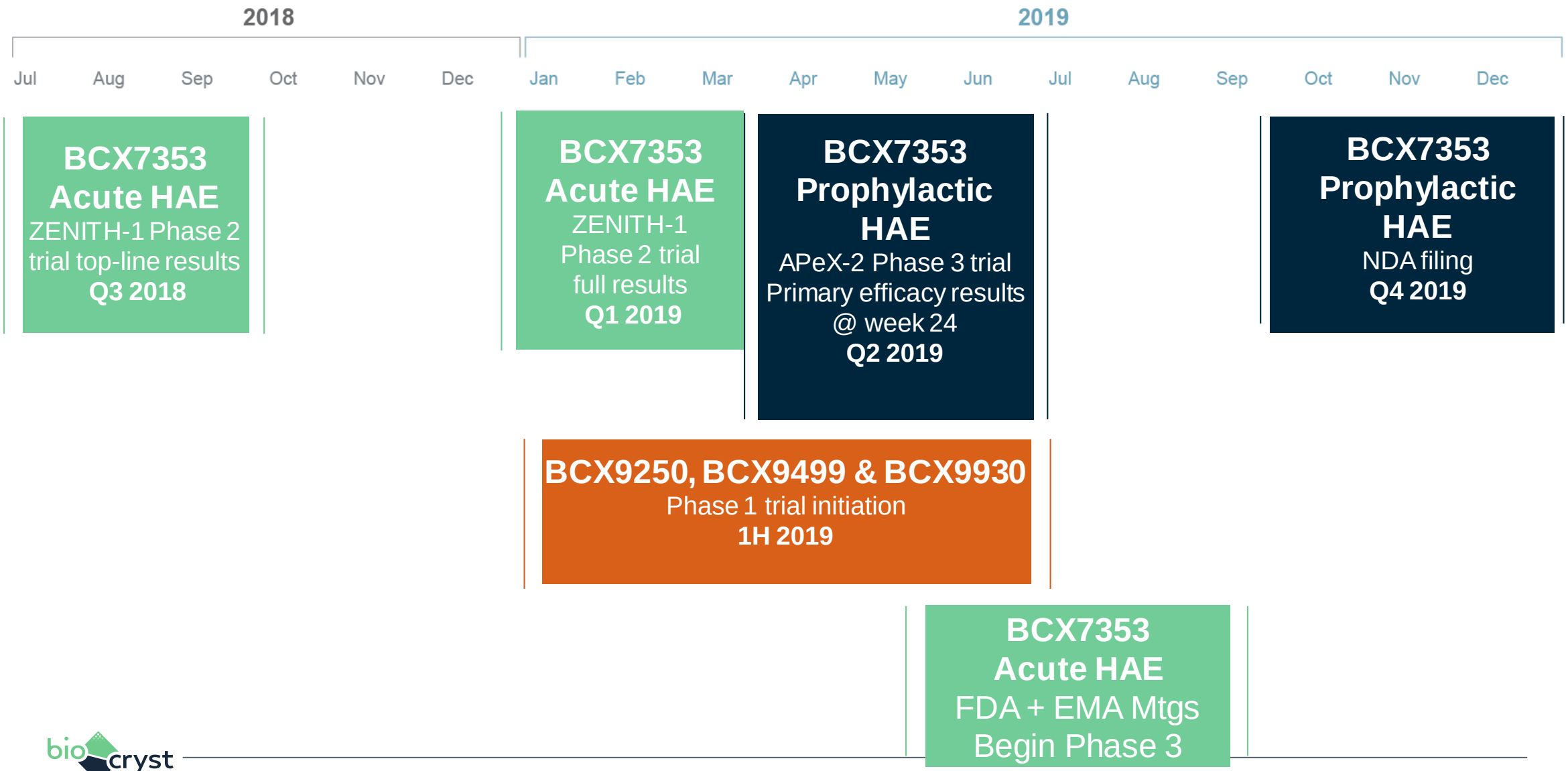
Agenda

- Update on Strategy and Pipeline: Several Near-term Milestones
  - Jon Stonehouse – President, Chief Executive Officer
- Clinical Update: APeX-2 Enrollment Complete, Data + NDA Timing On-track
  - Dr. Bill Sheridan – Chief Medical Officer
- Commercial Update: Patients Want an Oral Therapy
  - Lynne Powell – Chief Commercial Officer
- Financial Update: Recent Capital Infusion Funds Company into 2020
  - Thomas Staab – Chief Financial Officer
- Summary and Q&A

Update on Strategy and Pipeline: Several Near-term Milestones

Multiple Anticipated Milestones

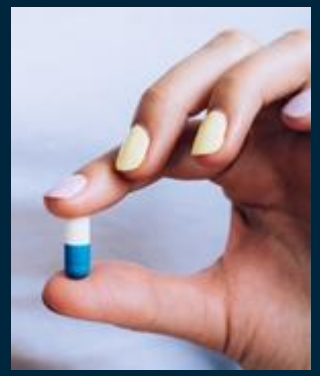


Clinical Update:
APeX-2 Enrollment Complete
Data + NDA Timing On-track

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

>$2 Billion global market opportunity

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

Regulatory Agency Status for BCX7353

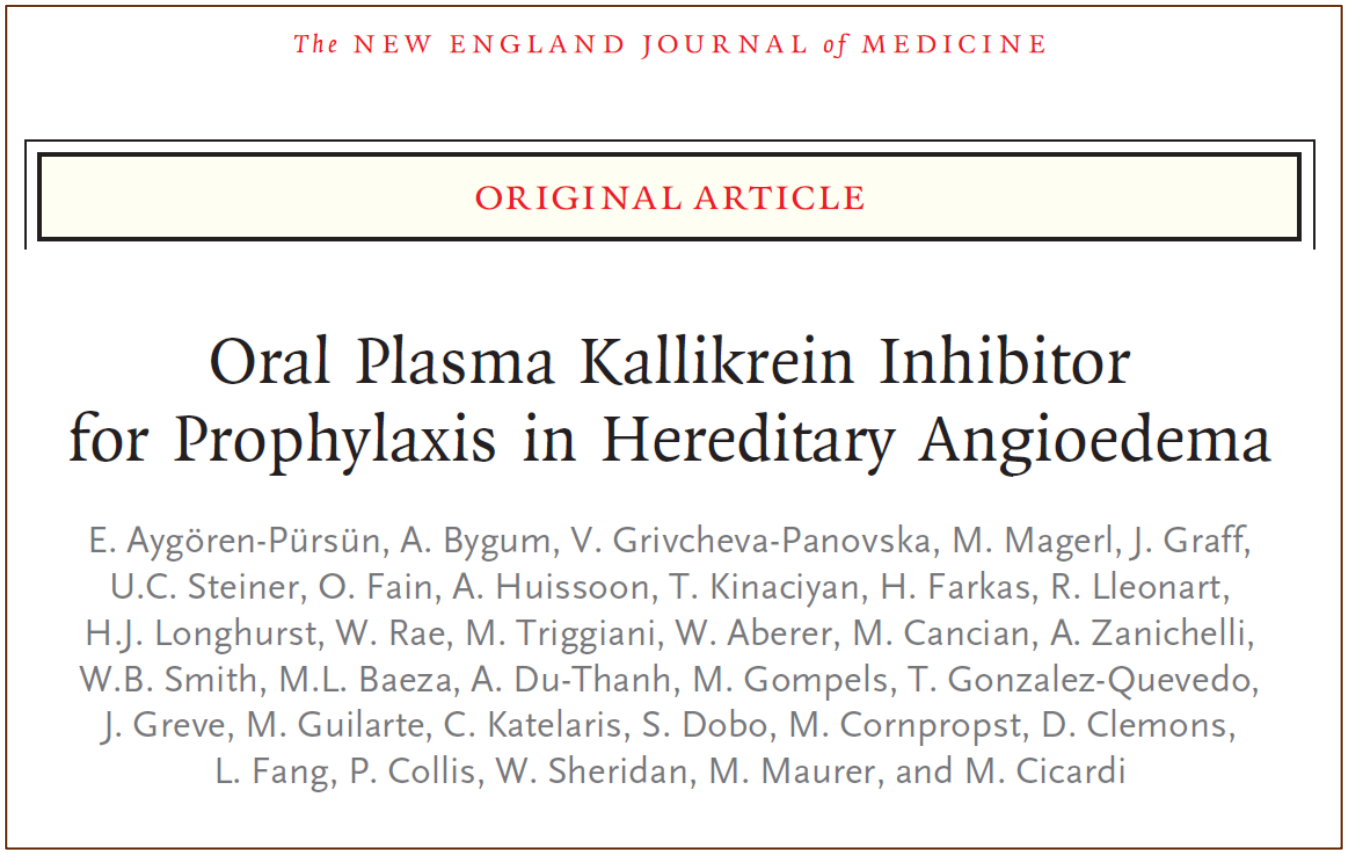
- Orphan Drug Designation 2017
- EOP2 2017
- Fast Track Designation

- 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 APeX-2 Trial Fully Enrolled



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

Blinded Treatment
24 weeks

N ≅ 32 BCX7353 150 mg QD*

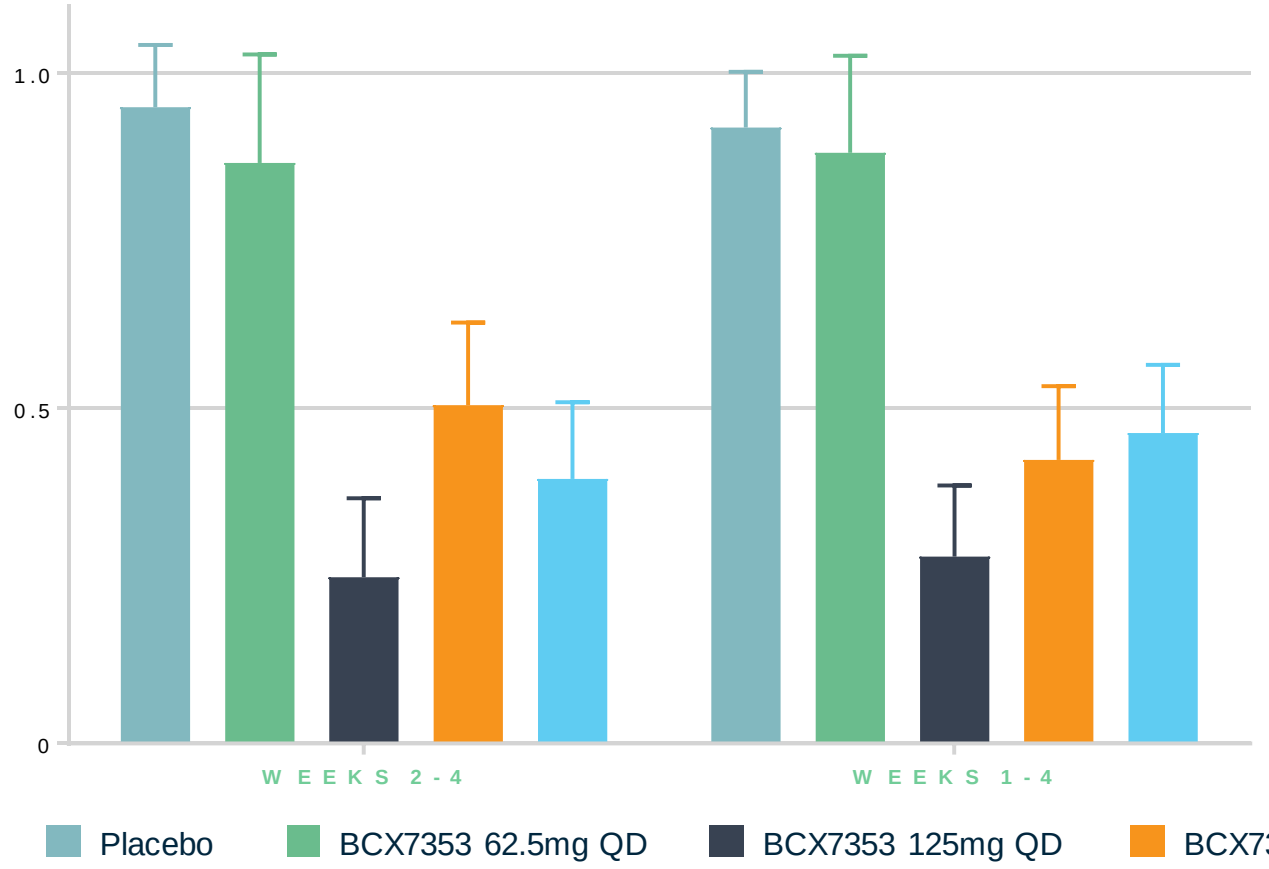
N ≅ 32 BCX7353 110 mg QD*

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

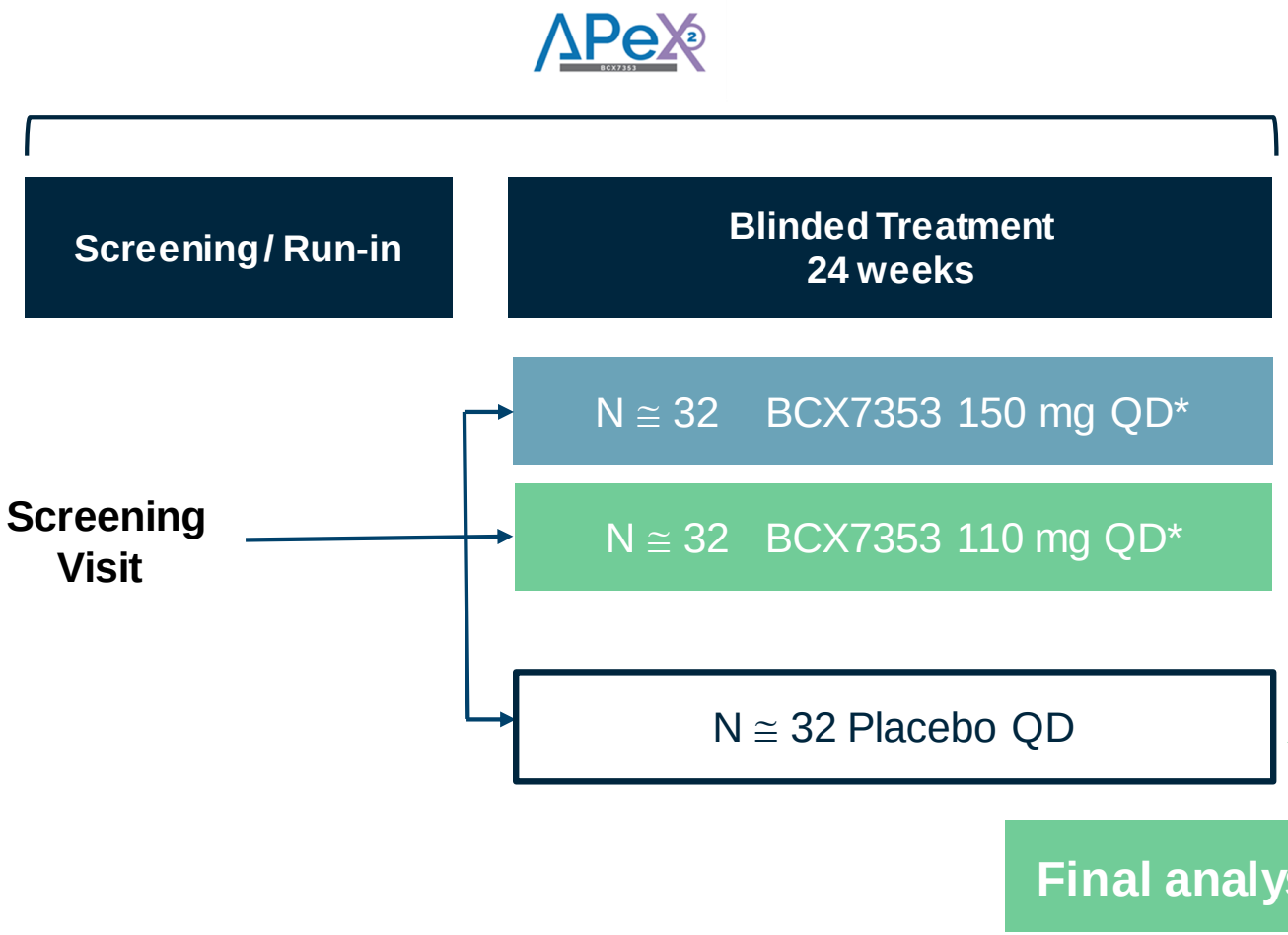
Final analysis

APeX-1: Treatment-Emergent Adverse Event Summary

Category	BCX7353				Placebo
	62.5 mg N = 7	125 mg N = 14	250 mg N = 14	350 mg N = 18	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.

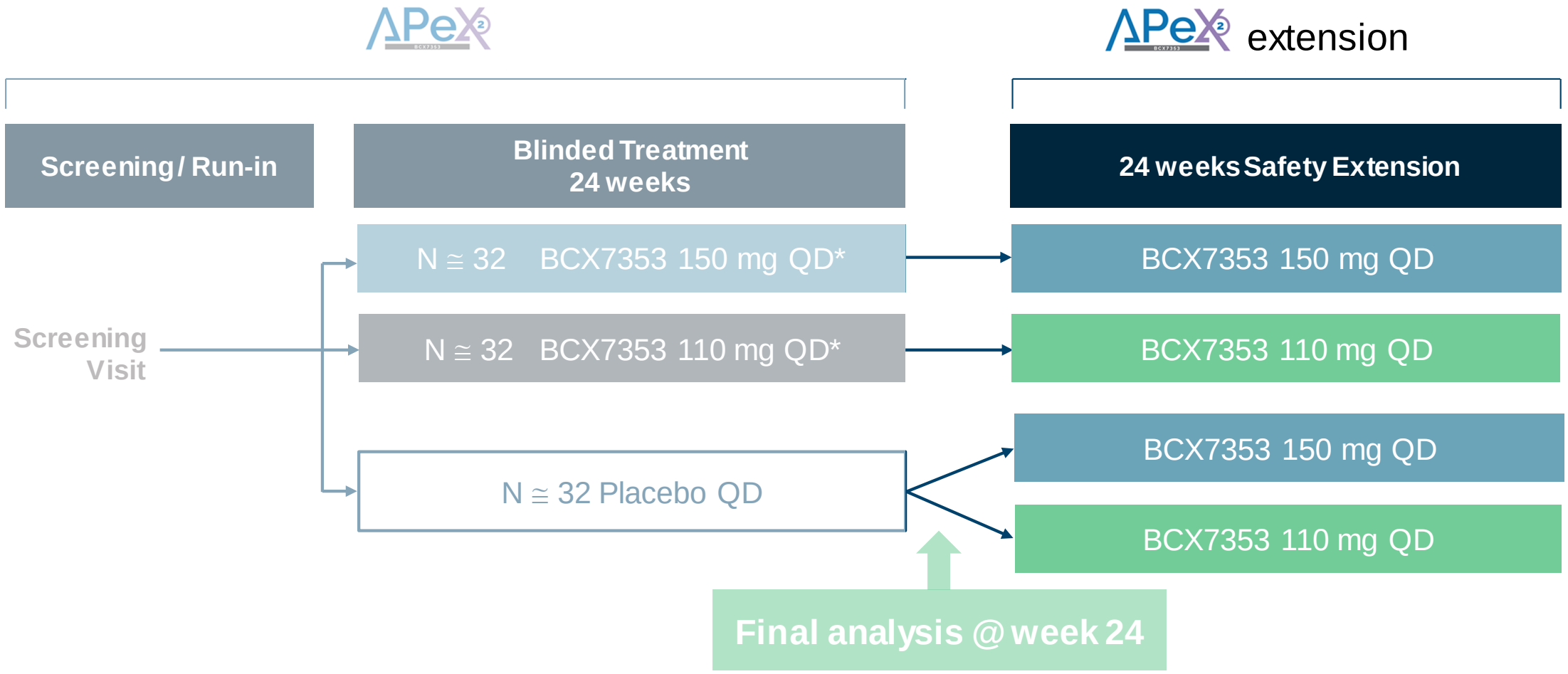
APeX-2: Phase 3 Trial Design



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

*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:
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

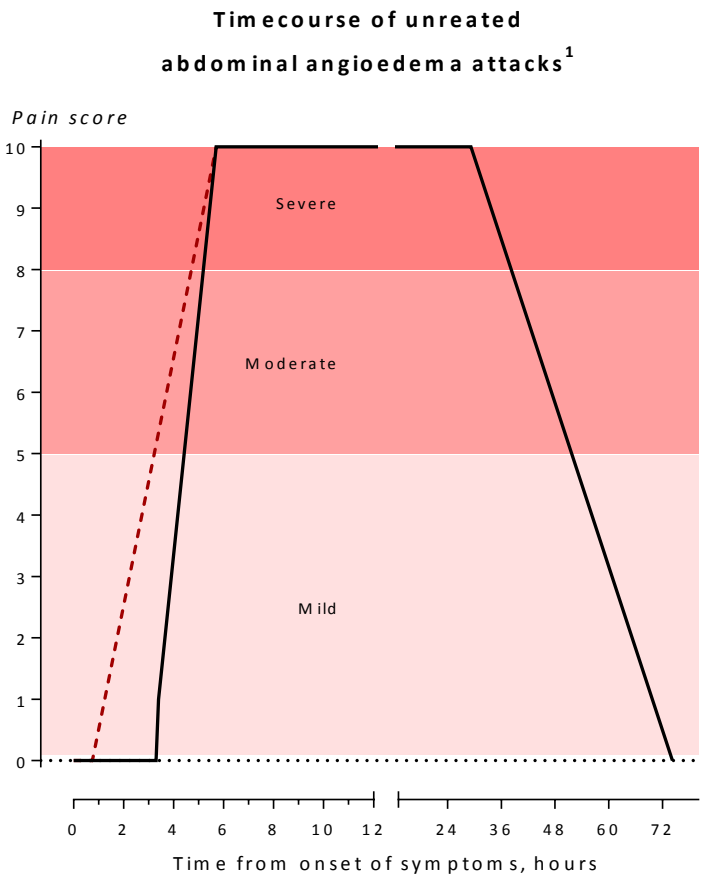
Analyses as needed for regulatory submissions

- 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

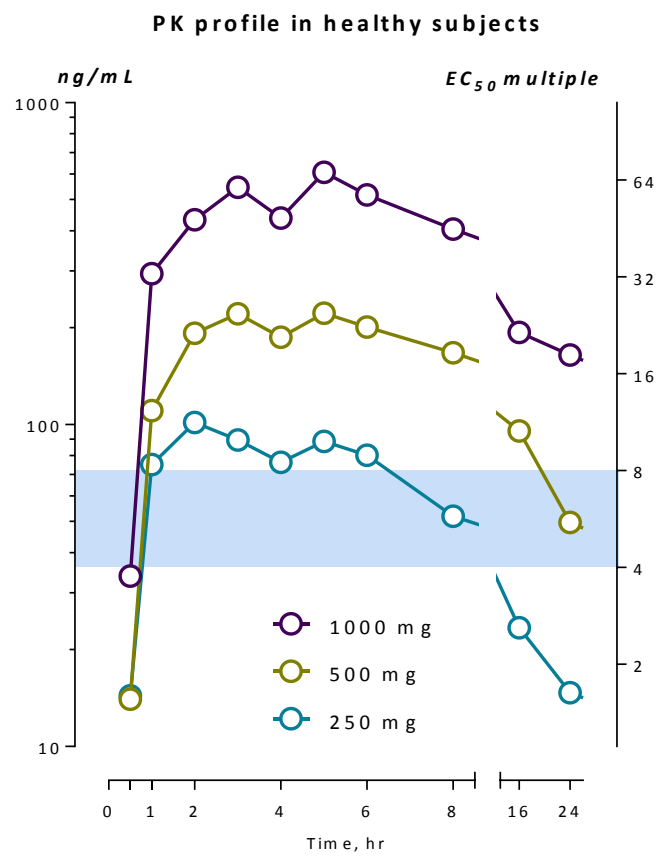
US Hereditary Angioedema Association Medical Advisory Board 2013 Recommendations for the Management of Hereditary Angioedema Due to C1 Inhibitor Deficiency

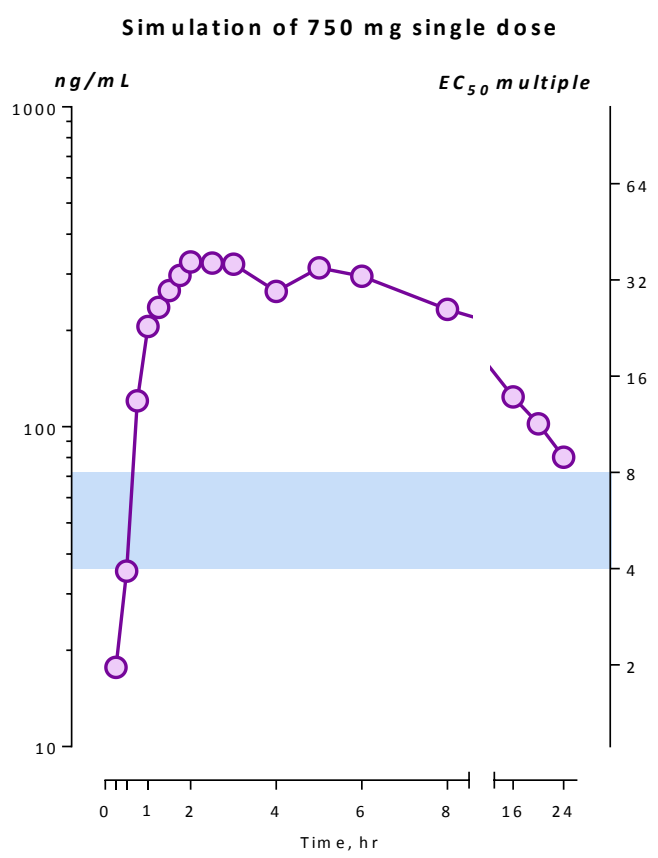
Bruce L. Zuraw, MD[a,b], Aleena Banerji, MD[c], Jonathan A. Bernstein, MD[d], Paula J. Busse, MD[e], Sandra C. Christiansen, MD[a,f], Mark Davis-Lorton, MD[g], Michael M. Frank, MD[h], Henry H. Li, MD[i], William R. Lumry, MD[j], and Marc Riedl, MD[k] *La Jolla, San Diego, and Los Angeles, Calif; Boston,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

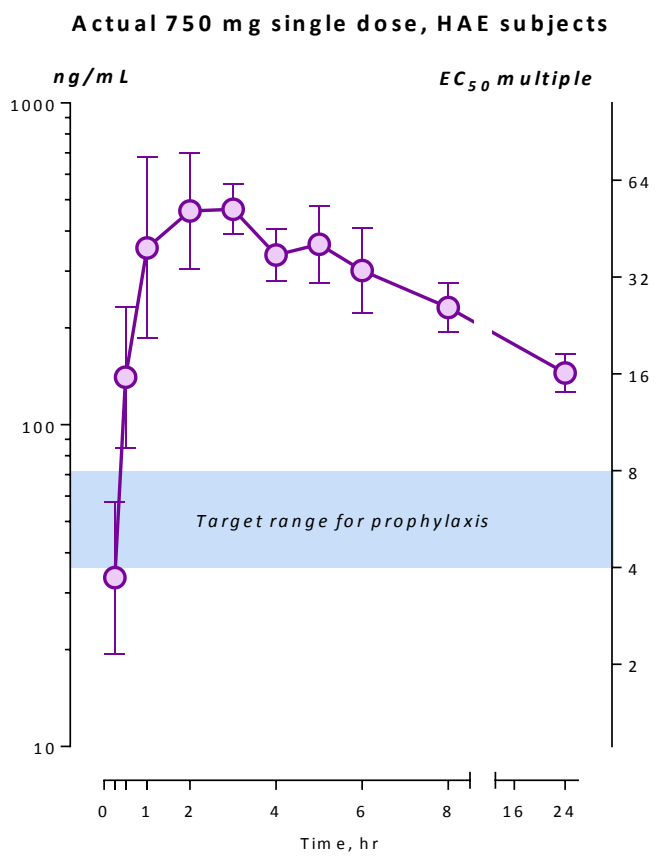
"On-demand treatment of attacks is most effective when administered early in the attack. Patients should be counseled to treat as soon as the attack is clearly recognized." [2]

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

PK Profile of Single Oral Dose of BCX7353 Supports Evaluation as an Acute Treatment in HAE







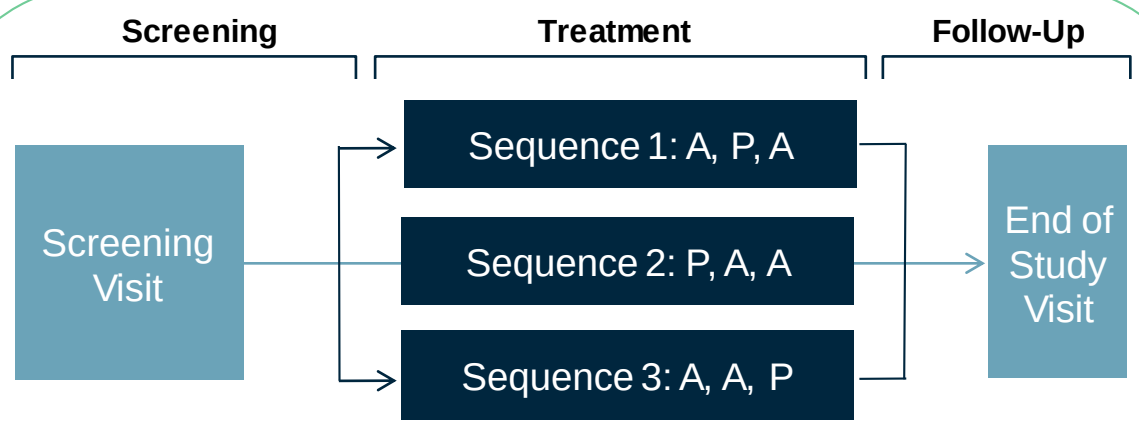
Data shown are means for healthy subjects and simulation and mean (SD) for HAE subjects

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 – Results for Part 1, 750 mg

Implementation successfully met the objective of early treatment after symptom onset



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*

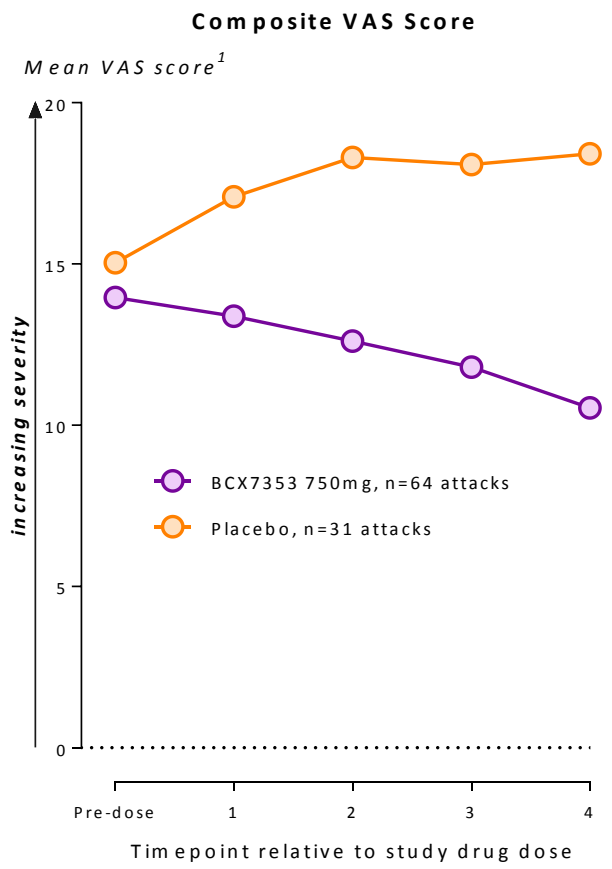
Subjects in Part 1	N
Subjects randomized	36
Subjects treated	33
Completed 3 on-study attacks	30

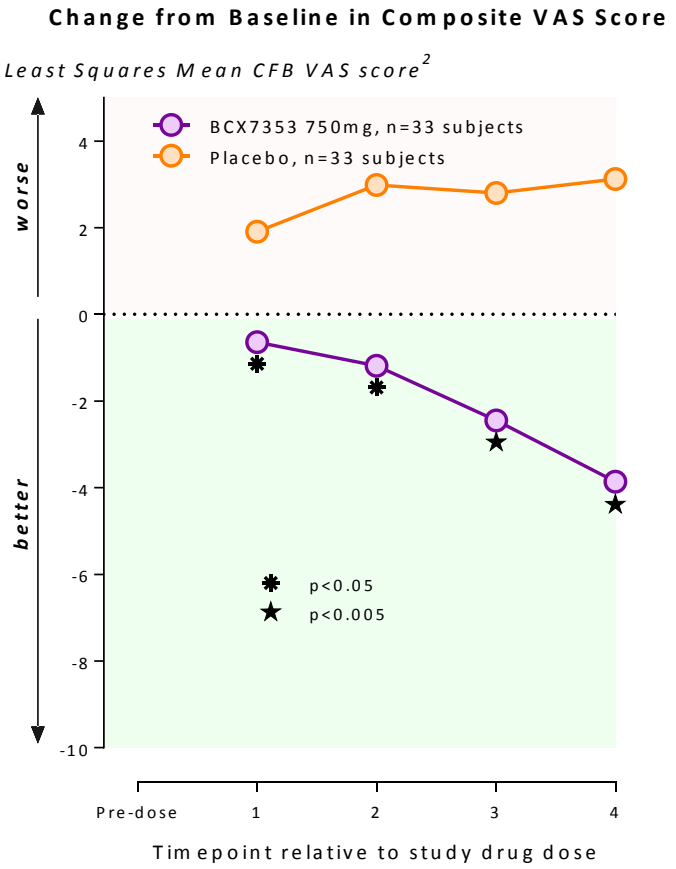
Attacks in Part 1	BCX7353	Placebo
Attacks treated, N	64	31
Time from onset of symptoms to study drug administration, min *median (range)*	35 (10-90)	35 (10-90)
Baseline mean VAS score	14	15

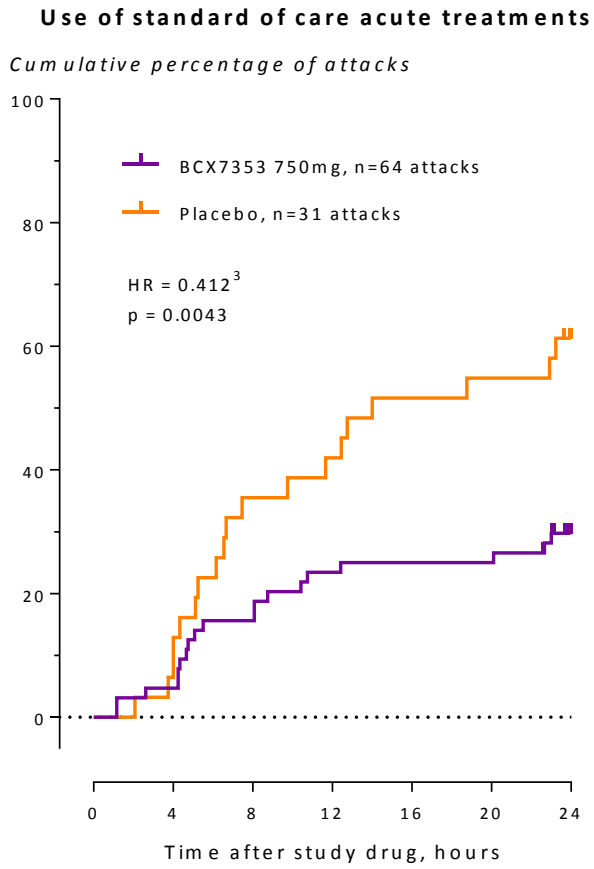
BCX7353 Showed Clinically Meaningful and Statistically Significant Benefits

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

Rapid and Sustained Benefit from BCX7353







Values after standard of care treatments are excluded.

[1] *The 3-symptom composite VAS was calculated as the average of three individual VAS scores of abdominal pain, cutaneous pain, and cutaneous swelling.*

[2] *Comparisons were performed separately at each time point using a mixed effect linear model including treatment, period and sequence as fixed effects, subject within sequence as a random effect, and predose 3-symptom composite VAS score as a covariate.*

[3] *Cox regression model for analysis of clustered data with time to event as the dependent variable and fixed effects for treatment, sequence and period. Subject was included in the model as a cluster variable.*

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.

BioCryst's Robust Pipeline

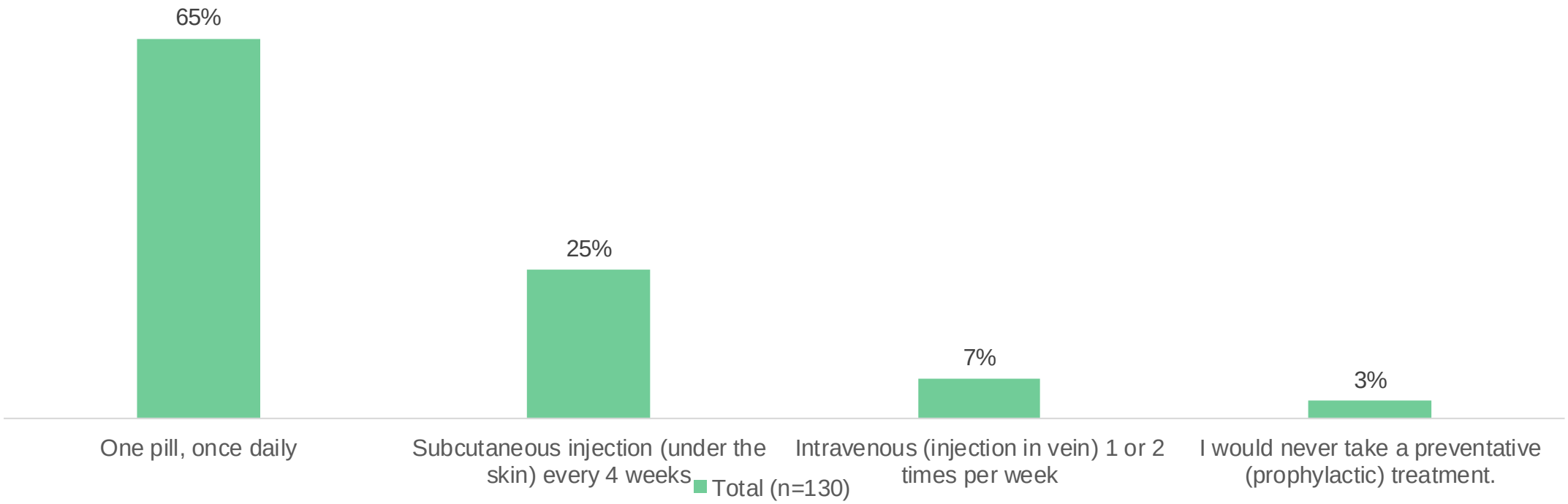
	Lead Optimization	Pre-clinical	Phase 1	Phase 2	Phase 3	Filed	Approved
STRATEGY: Develop oral therapies for life-threatening, rare diseases							
BCX7353 – Oral Capsule (Prophylactic HAE)							
BCX7353 – Oral Formulation (Acute HAE)							
BCX9250 & BCX9499 (FOP)							
Other rare diseases							
SUPPORTING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

*Licensed to Seqirus, Shionogi and Green Cross

Commercial Update:
Patients Want an Oral Therapy

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%

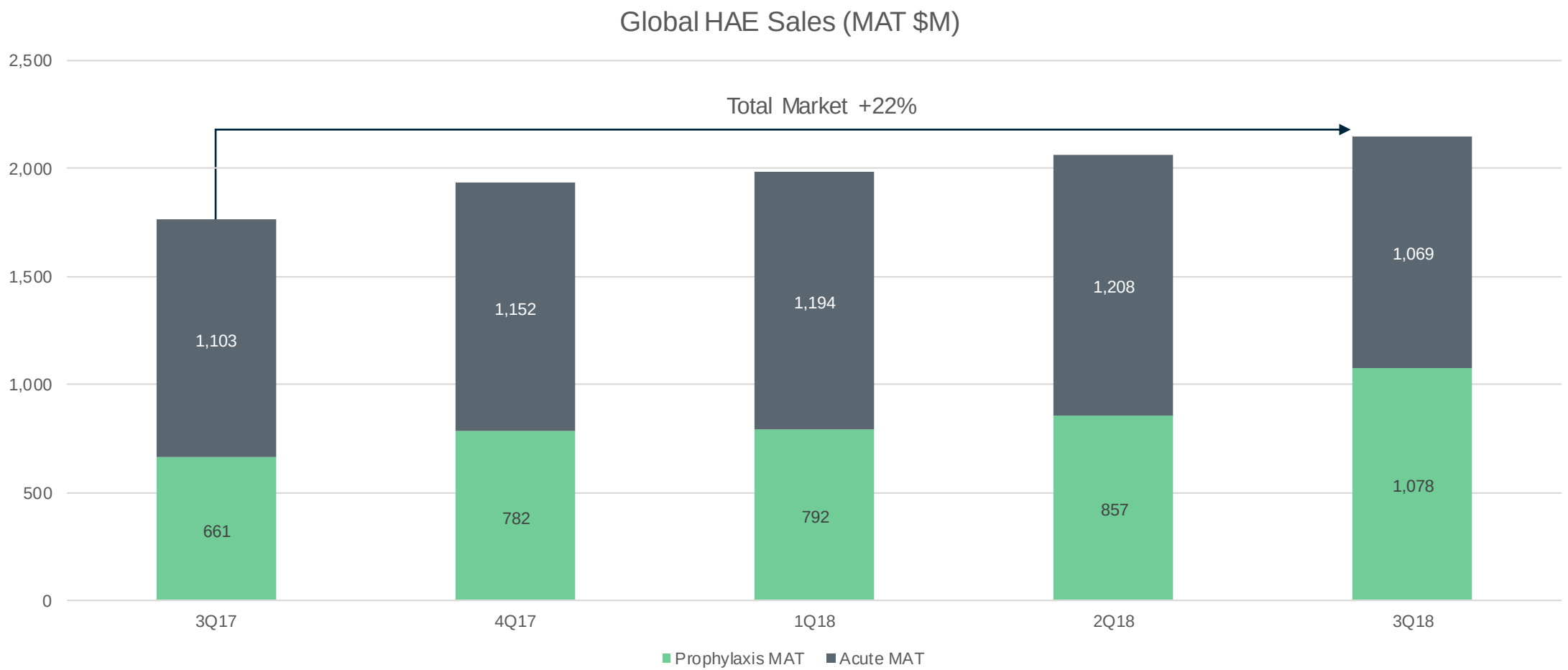
Unaided, cited ease of use/lack of injection as top reason

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 throug h Q3; and estimates for CSL products based on publicly reported data and comments in 2017 and 2018.

MAT= Moving 12 Month Average

Financial Update:
Recent Capital Infusion Funds Company into 2020

Third Quarter Operating Results

(in thousands, except per share amounts)	Q3 2018	Q3 2017	Change Q3 2018 vs Q3 2017
Revenues:			
Product sales	$ -	$ 1,501	(100%)
Royalty revenue	523	442	18%
Collaborative and other R&D	931	6,817	(86%)
Total revenues	1,454	8,760	(83%)
Expenses:			
Cost of product sales	-	1,142	(100%)
Research and development	22,006	17,509	26%
General and administrative	7,923	3,343	137%
Royalty	18	115	(84%)
Total operating expenses	29,947	22,109	36%
Loss from operations	(28,493)	(13,349)	113%
Interest and other income, net	611	225	172%
Interest expense	(2,346)	(2,140)	10%
Gain on foreign currency derivative	631	130	385%
Net loss	$ (29,597)	$ (15,134)	96%
Net loss per share - Basic & Diluted	$ (0.28)	$ (0.18)	56%
Net operating cash utilization	$ 29,407	$ 10,592	178%
Weighted average shares outstanding	105,410	83,570	

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.

Thank you…
Questions and Answers

November 6, 2018