Bank of America Merrill Lynch 2019 Health Care Conference

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Forward-Looking Statements

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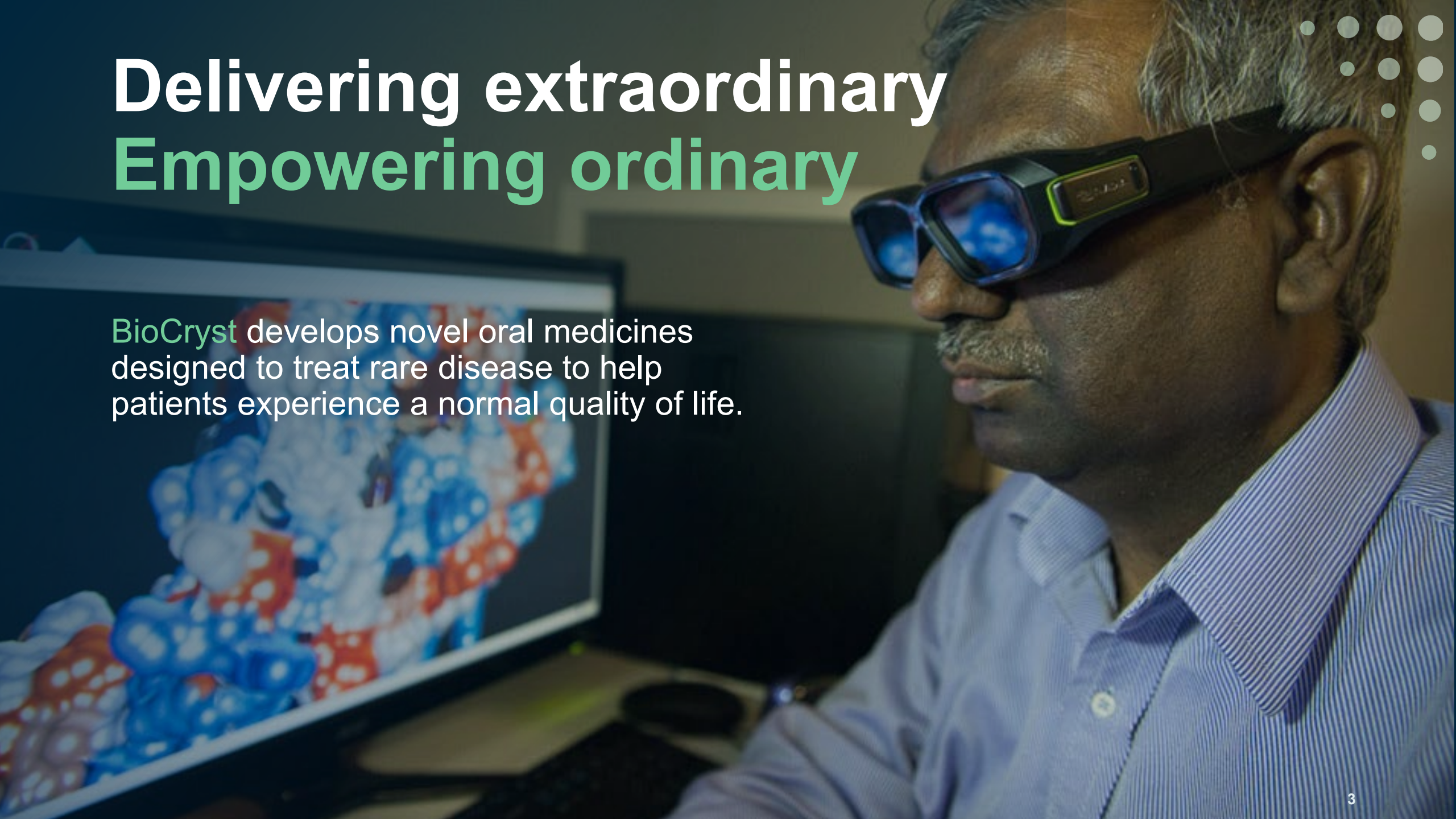
Delivering extraordinary
Empowering ordinary



BioCryst develops novel oral medicines designed to treat rare disease to help patients experience a normal quality of life.

BioCryst's Robust Pipeline

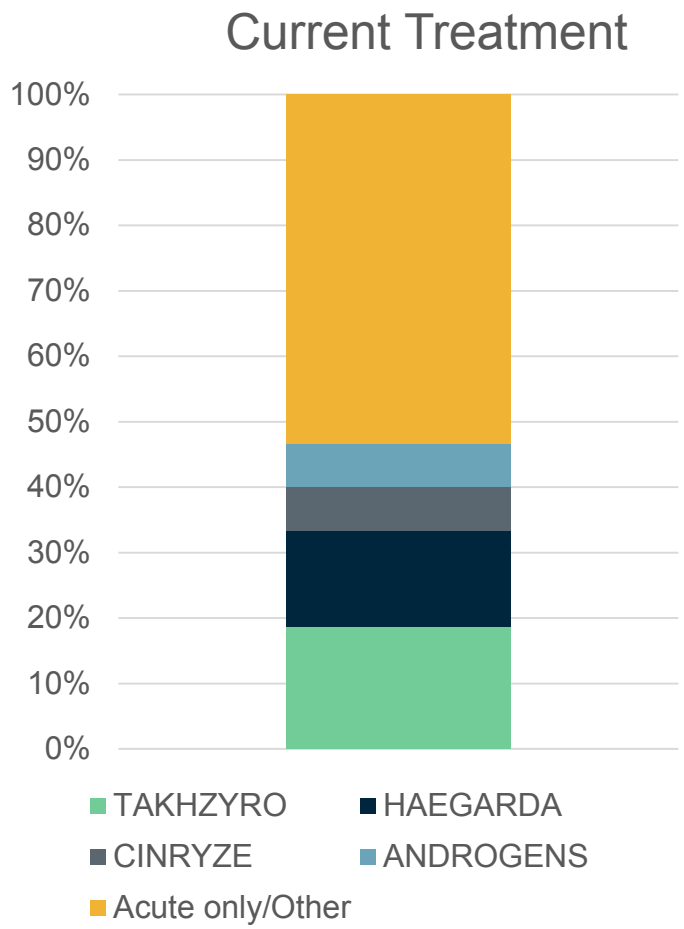
	Lead Optimization	Pre-clinical	Phase 1	Phase 2	Phase 3	Filed	Approved
STRATEGY: Develop oral therapies for life-threatening, rare diseases							
BCX7353 – Oral Capsule (Prophylactic HAE)							
BCX7353 – Oral Formulation (Acute HAE)							
BCX9930 – Oral Factor D Inhibitor Complement-Mediated Diseases							
BCX9250 (FOP)							
SUPPORTING ASSETS: Externally funded, potential for capital infusions							
RAPIVAB® (peramivir injection)*							
Galidesivir (Broad spectrum antiviral) I.V.							

*Licensed to Seqirus, Shionogi and Green Cross

BioCryst HAE Prophylactic Program (BCX7353)

HAE Patients Really Want Oral Prophylaxis

US HAE patient survey fielded November 2018 (n=75)



*An oral preventative HAE medication **would fit my life better** than an injectable HAE medication*

97% agree

*I like my current preventative HAE medication, but if an oral preventative HAE medication became available, **I would switch** to that new medication**

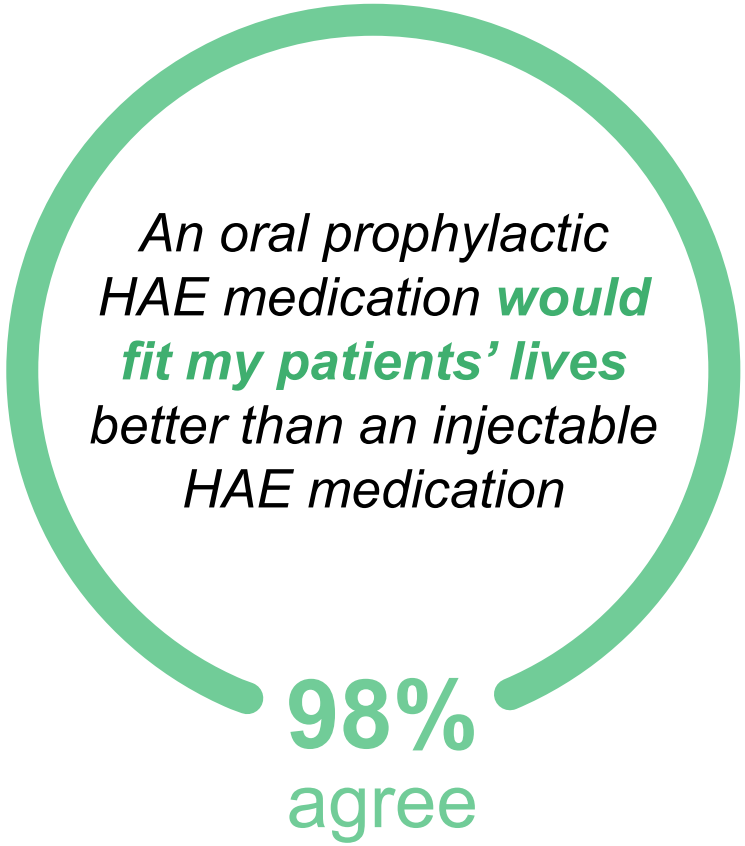
89% agree

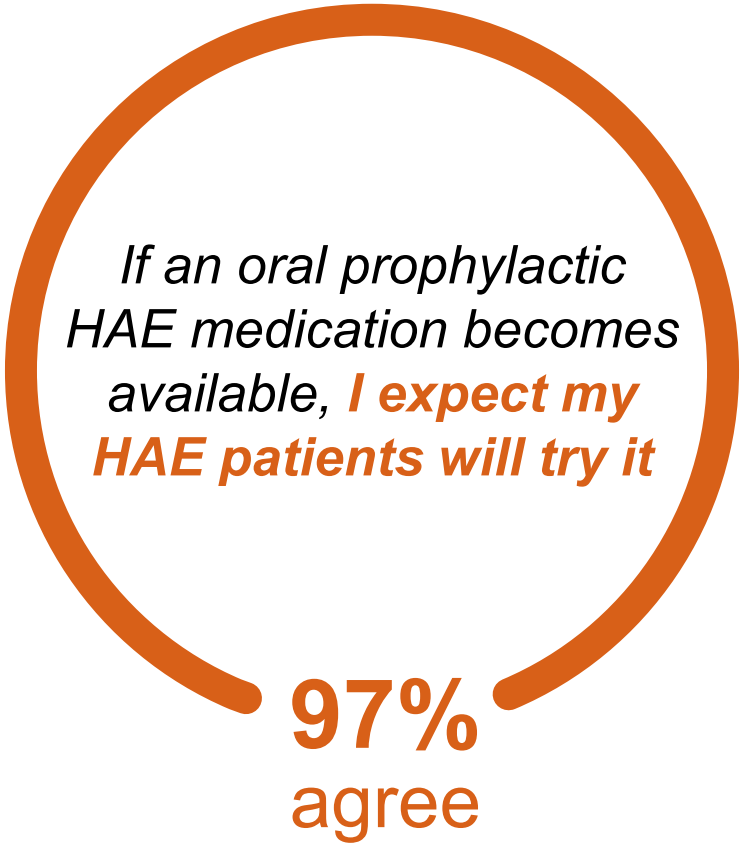
*10 out of 14 patients on TAKHZYRO agreed with this statement

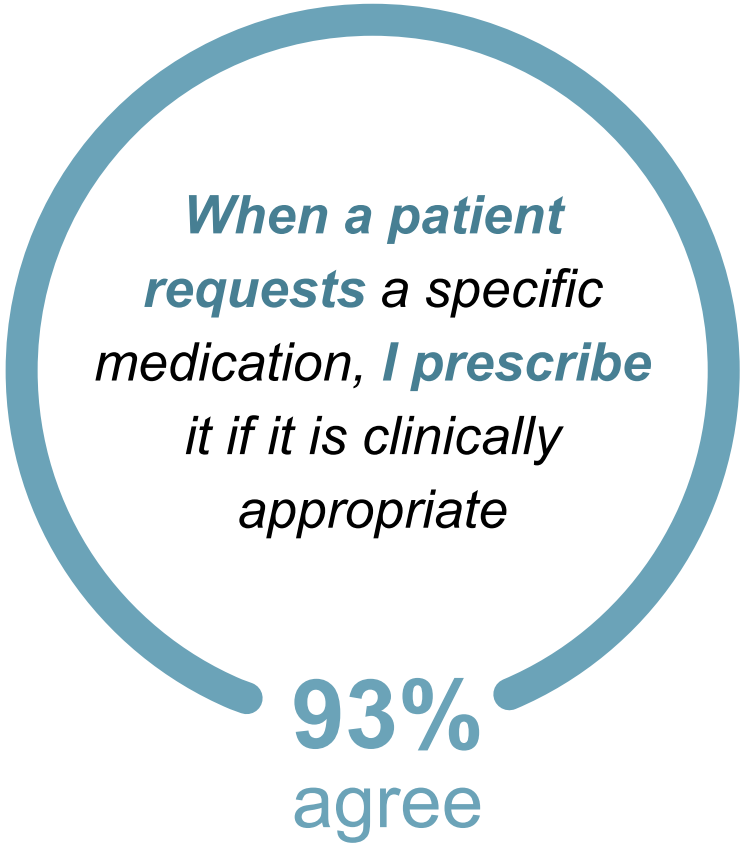
ALL QUALIFIED RESPONDENTS
Q600-609 Please read the following statement and indicate if you agree or disagree.

Allergists Understand what HAE Patients Want

US allergist survey: November 2018 (n=100)

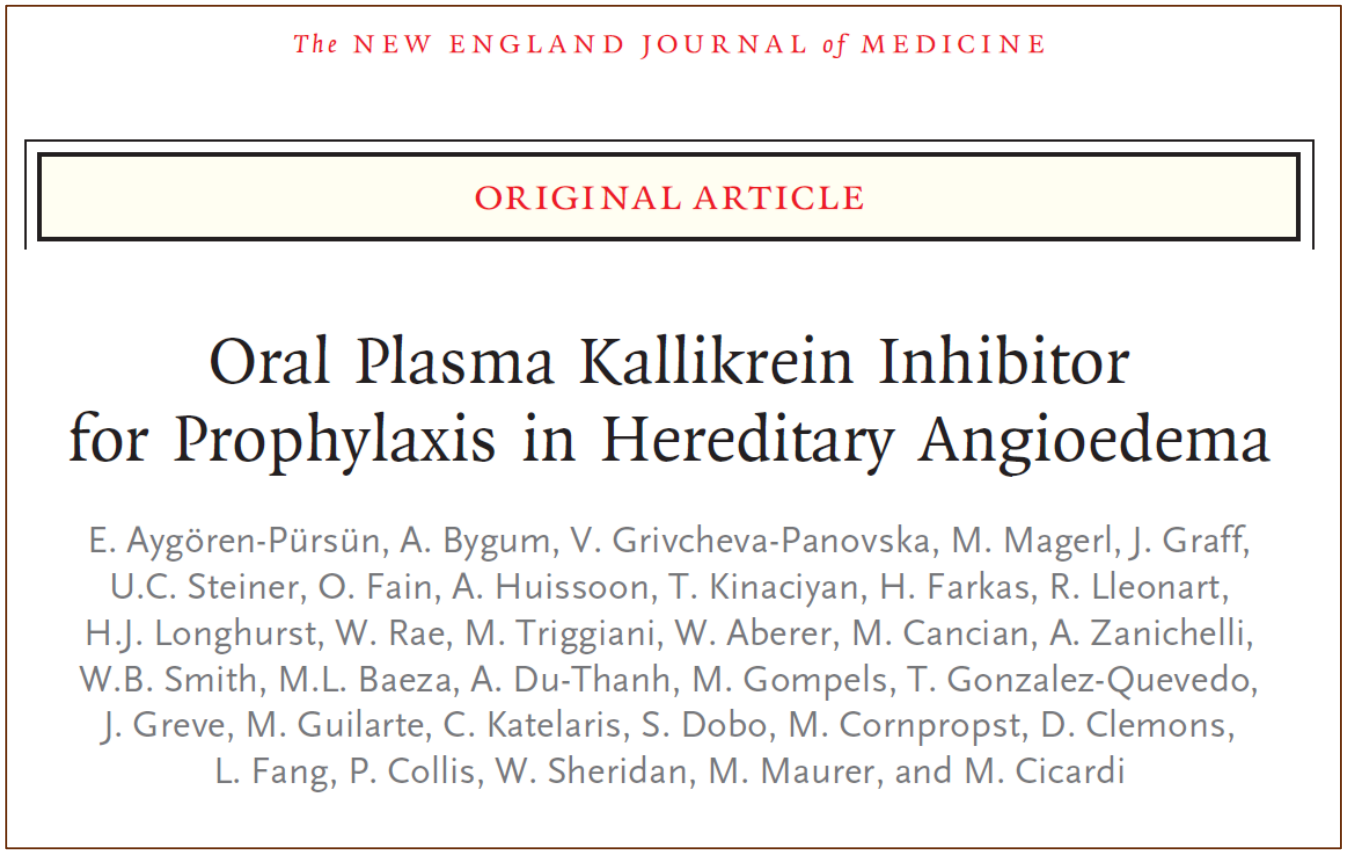






ALL QUALIFIED RESPONDENTS
Q600-609 Please read the following statement and indicate if you agree or disagree.

BCX7353 Phase 2 APeX-1 Trial Published, Phase 3 APeX-2 Trial Fully Enrolled



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 BCX7353

Blinded Treatment
24 weeks

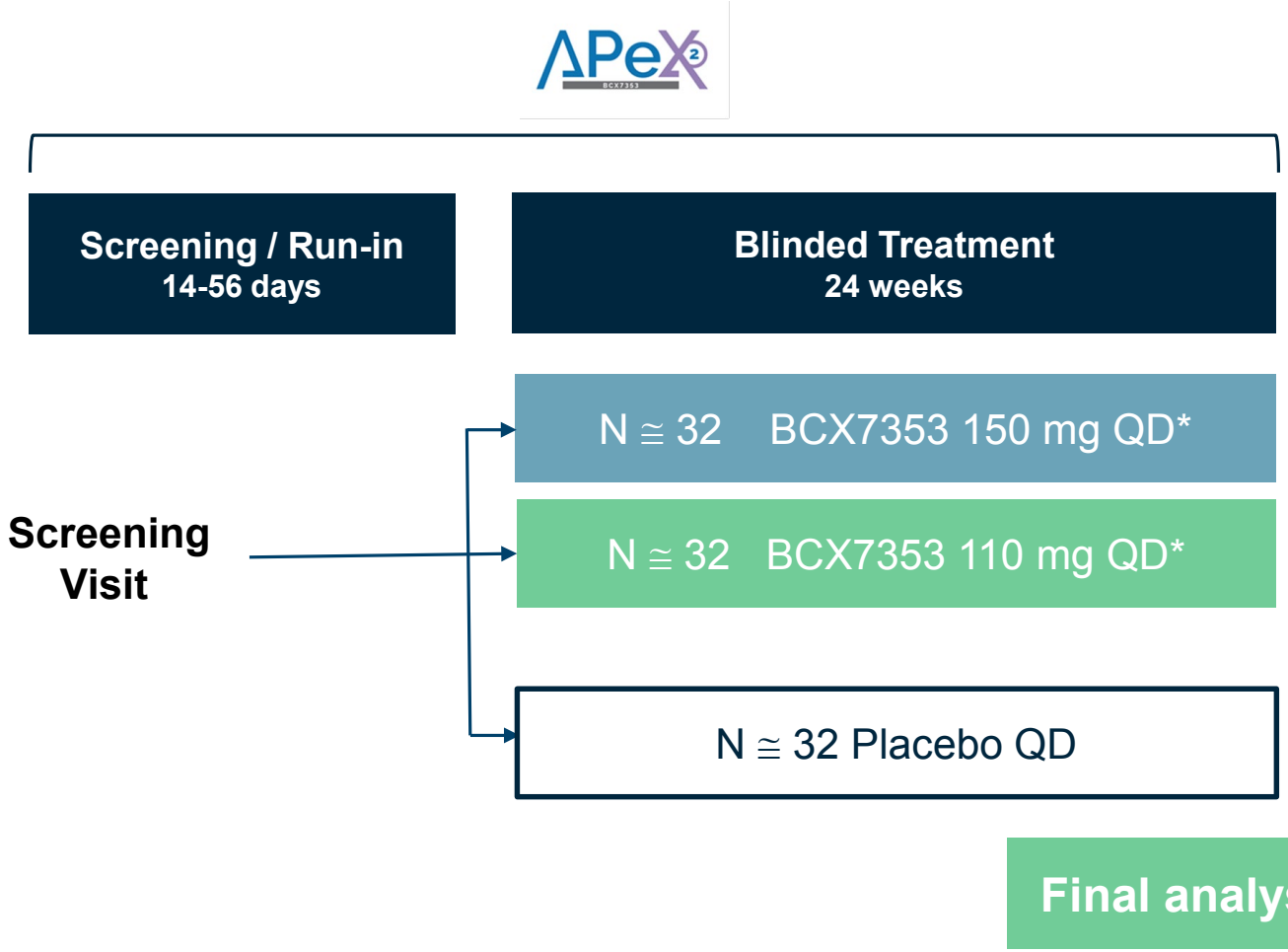
N ≅ 32 BCX7353 150 mg QD*

N ≅ 32 BCX7353 110 mg QD*

N ≅ 32 Placebo QD

Final analysis @ week 24

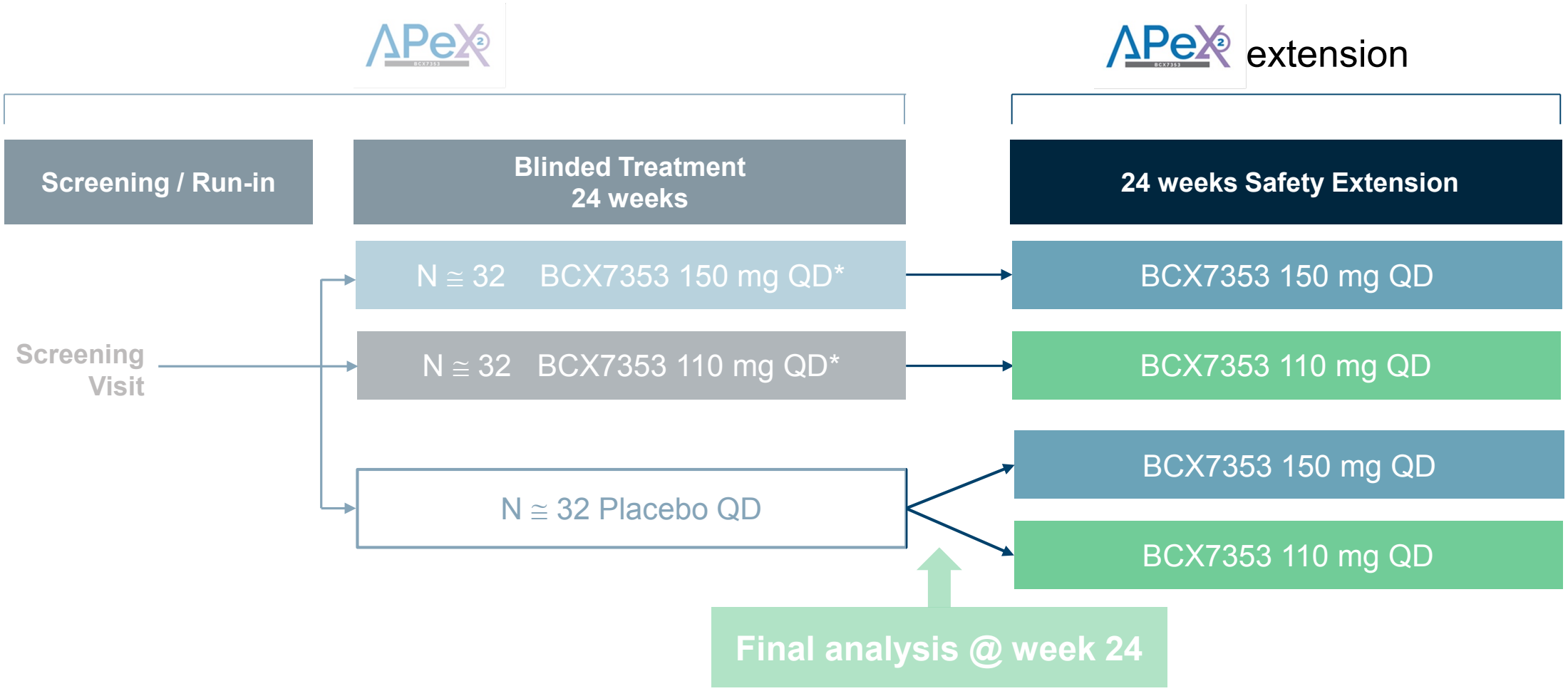
APeX-2: Phase 3 Trial Design



- 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%

*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

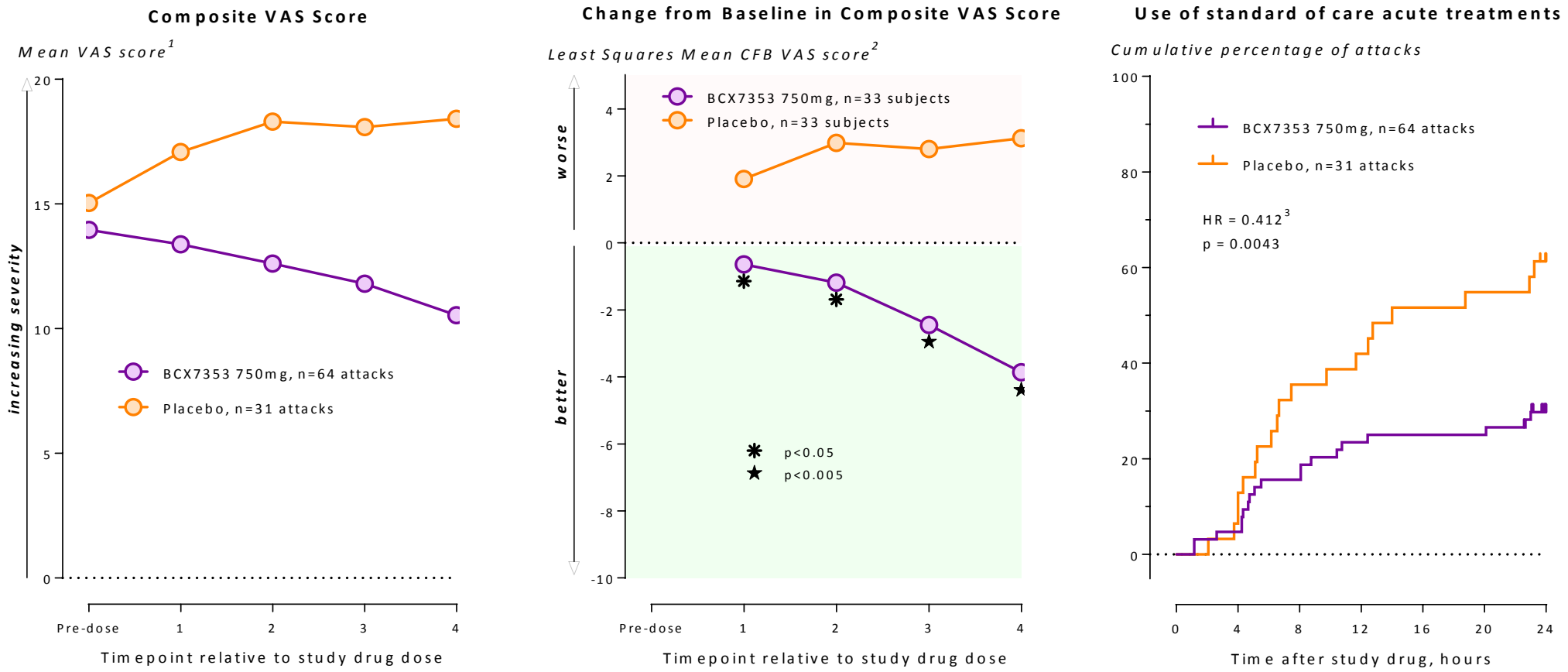
- Endpoints:
  - Long term safety of BCX7353
  - Durability of response
  - Quality of Life
- APeX-1 subjects eligible

- 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

BioCryst HAE Acute Treatment Program (BCX7353)

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.*

ZENITH-1 – Safety Summary

	BCX7353			All Placebo
	750 mg	500 mg	250 mg	
Number of subjects treated with at least 1 dose of study drug	33	14	11	53
Number of attacks treated*	64	25	21	53
Number of attacks with a reported treatment-emergent adverse events (TEAE)	16 (25.0%)	10 (40.0%)	10 (47.6%)	17 (32.0%)
Number of attacks with a serious TEAE ¥	0	1 (4.0%)	0	1 (1.9%)
Number of attacks with a drug-related TEAEs as assessed by investigator	7 (10.9%)	5 (20.0%)	6 (28.6%)	6 (11.3%)
Number of attacks with TEAEs leading to permanent discontinuation from study drug	1 (1.6%) ‡	1 (4.0%)€	0	1 (1.9%) §
Number of attacks with TEAEs of Grade 3 or Grade 4	0	1 (4.0%)Δ	0	0
Number of attacks with TE lab abnormalities of Grade 3 or 4	0	0	0	0
Number of attacks with drug-related TEAEs of Grade 3 or 4	0	0	0	0
Most common adverse events				
Diarrhea	3 (4.7%)	3 (12.0%)	0	2(3.8%)
Abdominal pain	2 (3.1%)	3 (12.0%)	1 (4.8%)	1 (1.9%)
Nausea	2 (3.1%)	2 (8.0%)	2 (9.5%)	0
Nasopharyngitis	4 (6.3%)	0	0	1 (1.9%)
Headache	3 (4.7%)	0	3 (14.3%)	1 (1.9%)

* 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.

Δ Grade 3 serious TEAE of Ankle fracture

‡ 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.

¥ The serious TEAEs of Motorvehicle accident and Ankle fracture were not drug-related.

€ Discontinuation on BCX7353 occurred in a subject who experienced moderate vomiting and nausea.

BioCryst Oral Factor D Inhibitor (BCX9930)

BCX9930 a Potent and Selective Inhibitor of Factor D

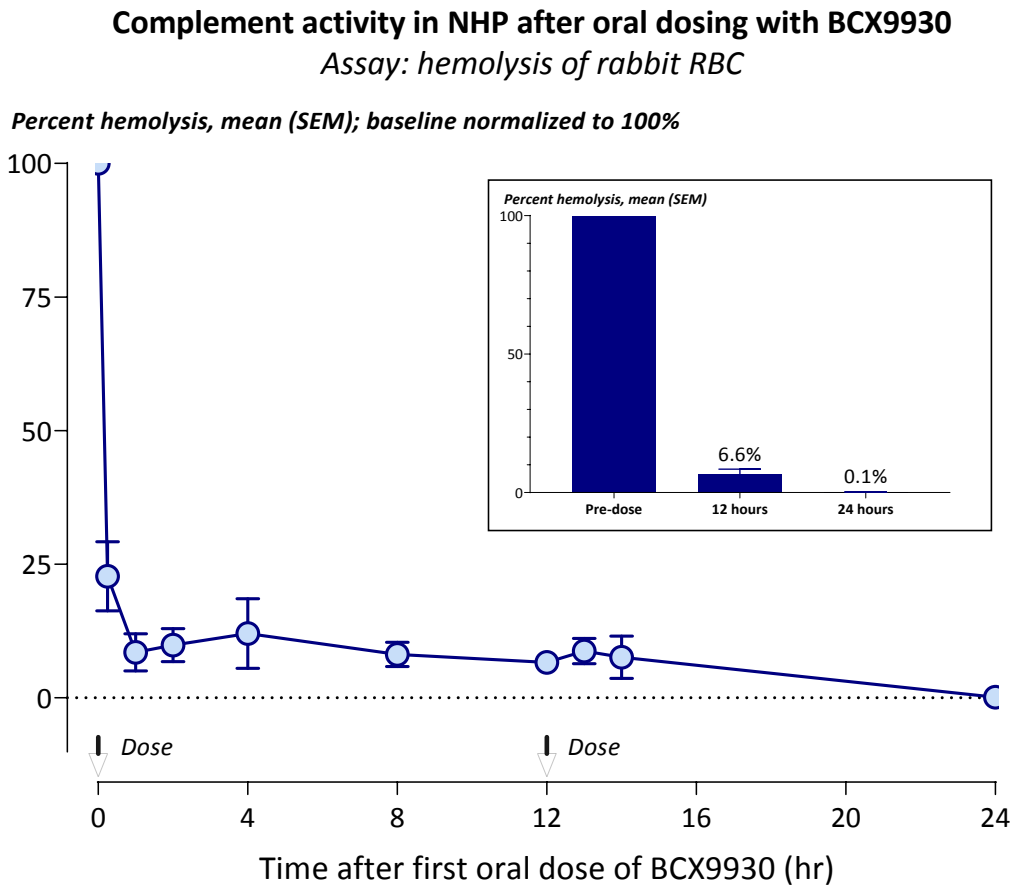
Potency Assays

Assay	Mean IC_{50} or EC_{50}, nM
Factor D esterolytic activity	≈ 15
Cleavage of complement enzyme C3bB by Factor D	≈ 30
Hemolysis of rabbit RBC by human serum	≈ 30
Acid-induced complement-mediated hemolysis of PNH patient RBC	≈ 30
Complement enzyme C3 deposition on PNH patient RBC incubated with acidified C5-deficient serum	≈ 40

Specificity Assays

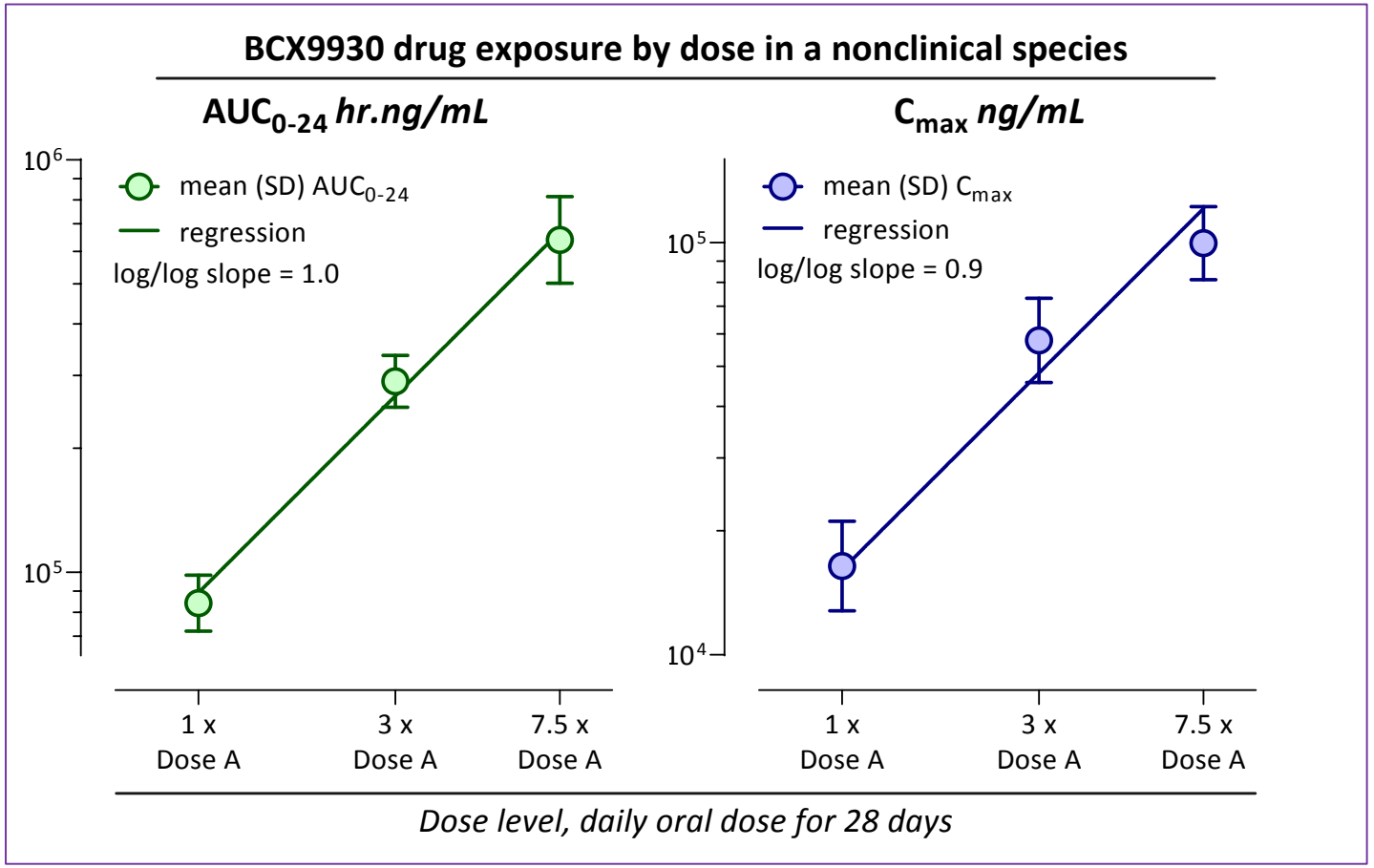
Serine Proteases	Selectivity Ratio relative to Factor D
Complement enzyme C1s	>60
Plasmin	≈ 200
Thrombin	>2000
Activated protein C	>2000
Tissue plasminogen activator	>2000
Trypsin	>2000
Factor Xa	>3000
Factor XIIa	>3000

BCX9930 Inhibits Complement-Mediated Hemolysis in Standard Ex-Vivo Assay After Oral Dosing in NHP



- Hemolysis of rabbit RBC by serum is a very well-established assay, originally developed to detect complement deficiency
- After oral dosing of NHPs with BCX9930, >99.9% suppression of complement-mediated hemolysis was observed
- Drug exposure (AUC_{0-24}) in this experiment was a fraction of the NOAEL
- BCX9930 is approx. 50% *less* potent on NHP compared with human Factor D

Wide Preclinical Safety Margin Provides Significant Dosing Flexibility for Clinical Trials



- High drug levels after oral dosing in 2 nonclinical species
- Linear and dose-proportional exposure in nonclinical species
- Very high NOAELs: human equivalent dose = approx. >5,000 mg
- Large safety margins for entry into the clinic:
  - C_{max} at NOAELs more than 500 times the estimated therapeutic target level

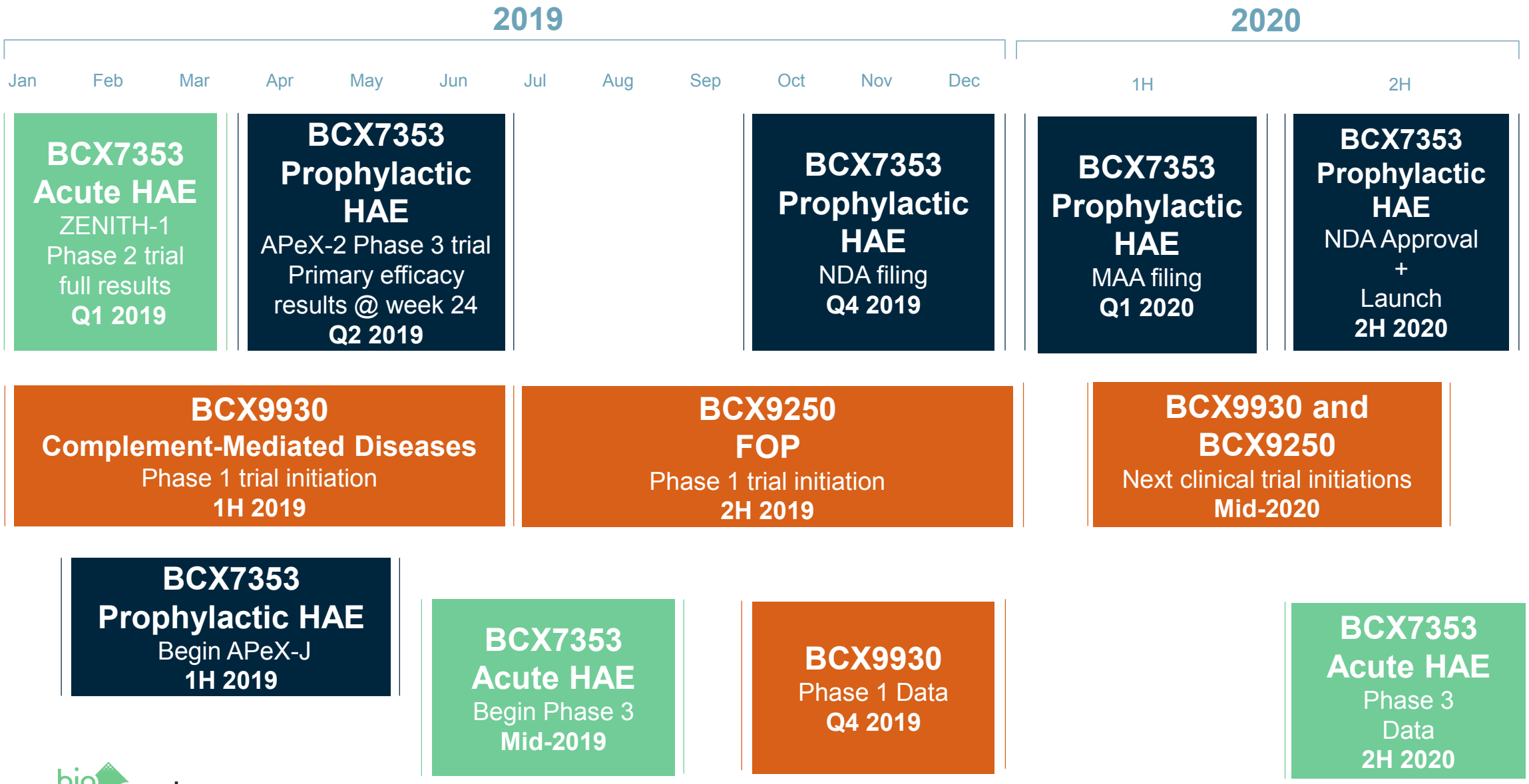
Cash position & 2019 guidance (in millions)

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.

Many Anticipated Milestones in 2019 - 2020



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