Barclays Global Healthcare Conference

Jon Stonehouse

Chief Executive Officer

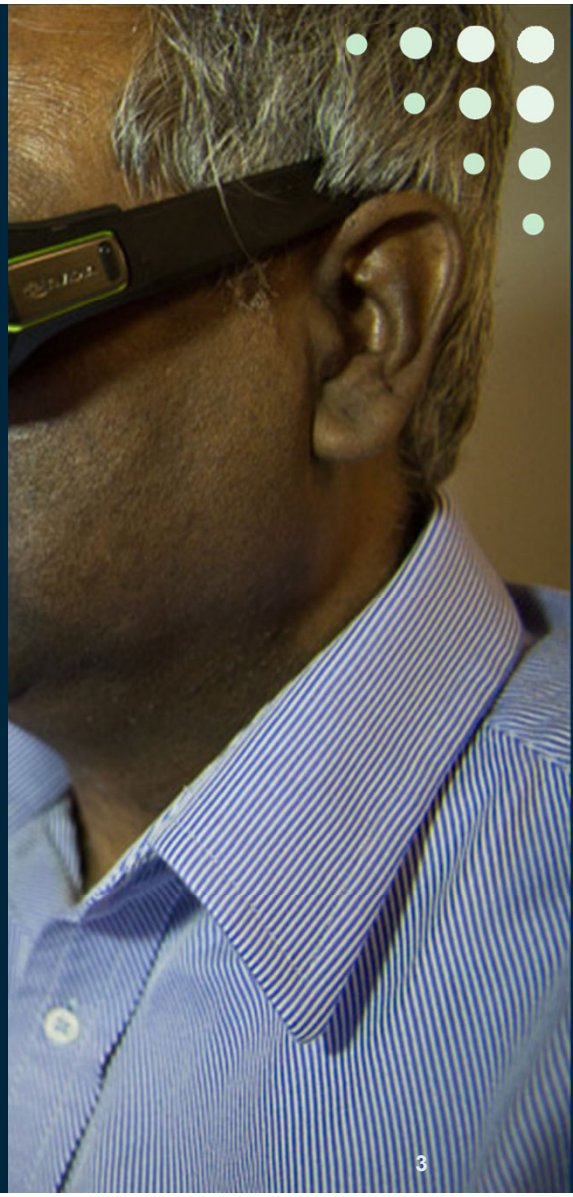
March 12, 2019

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 and located at http://investor.shareholder.com/biocryst/sec.cfm

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

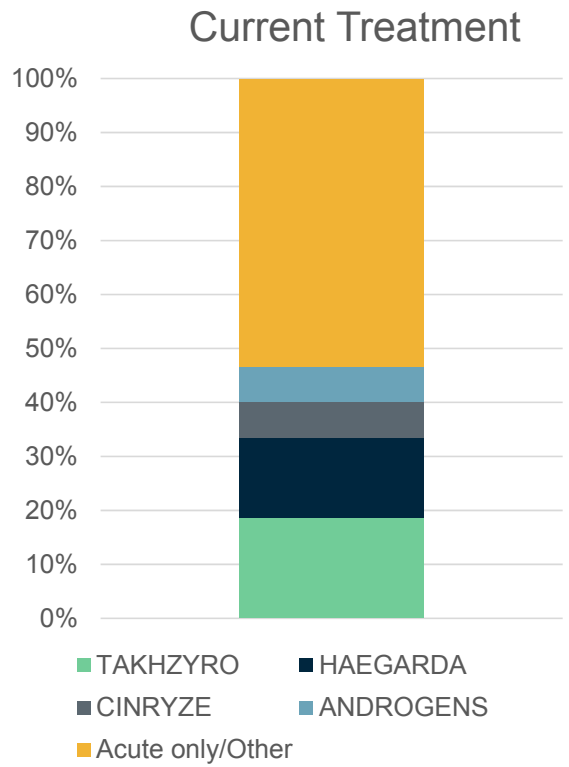
	Lead Optimization	Pre-clinical	Phase 1	Phase 2	Phase 3	Filed	Approved
STRATEGY: Develop oral therapies for life-threatening, rare diseases							
BCX7353 – Oral Capsule (Prophylactic HAE)							
BCX7353 – Oral Formulation (Acute HAE)							
BCX9930 – Oral Factor D Inhibitor Complement-Mediated Diseases							
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**

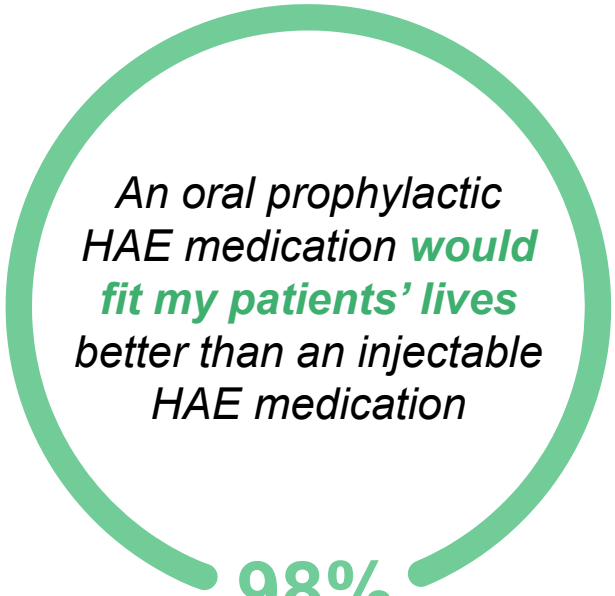
89% agree

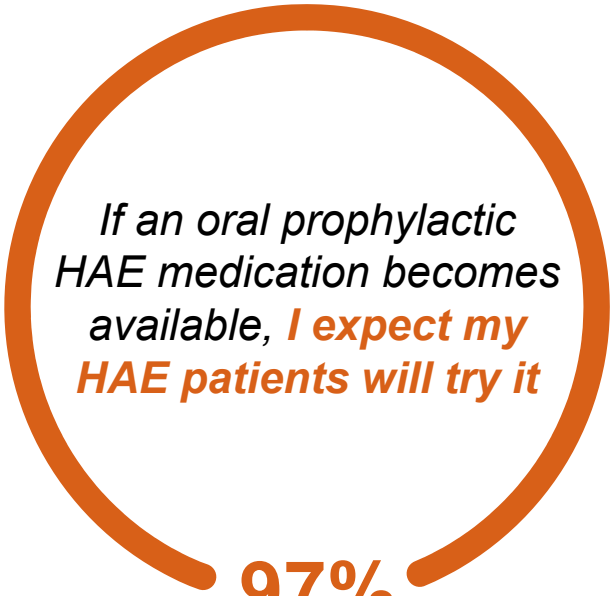
*10 out of 14 patients on TAKHZYRO agreed with this statement

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

Allergists Understand what HAE Patients Want

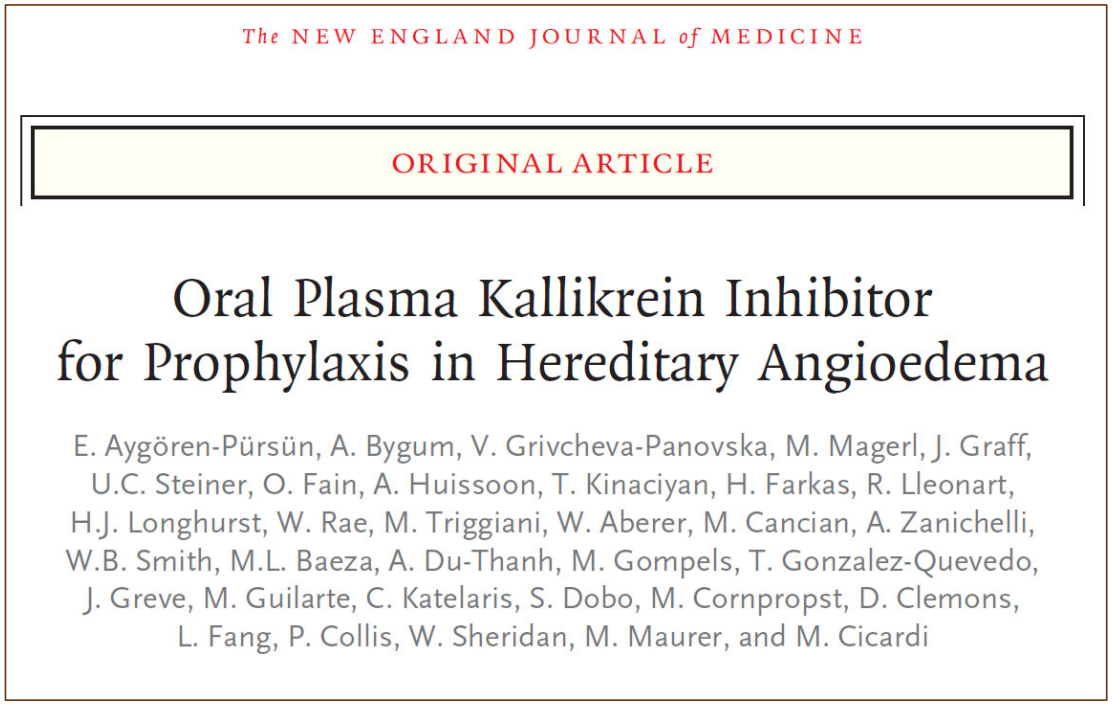
US allergist survey: November 2018 (n=100)





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

BCX7353 Phase 2 APeX-1 Proof of Concept Trial



The NEW ENGLAND JOURNAL of MEDICINE

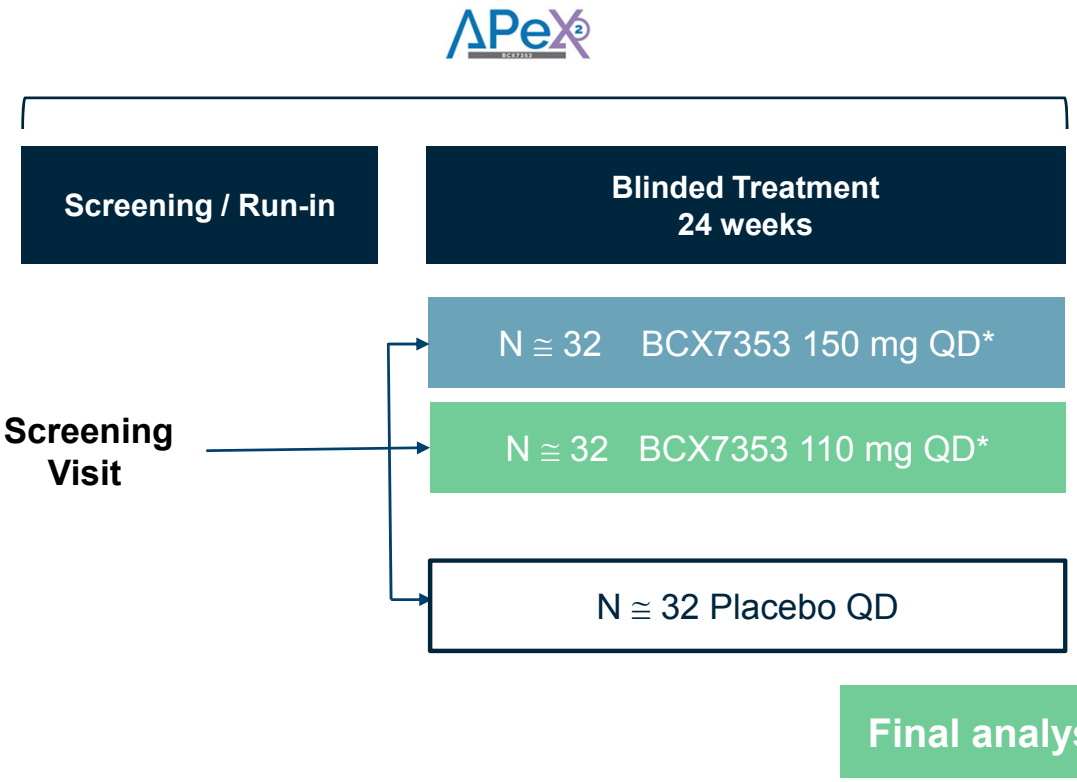
ORIGINAL ARTICLE

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: 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

Substantial Increase in Patient Experience in Past 12 Months

End of 2017 (APeX-1)

53 patients with up to **4 weeks** exposure at doses of 62.5 – 350 mg

~4 WEEKS

End of 2018 (APeX-2 and APeX-S)

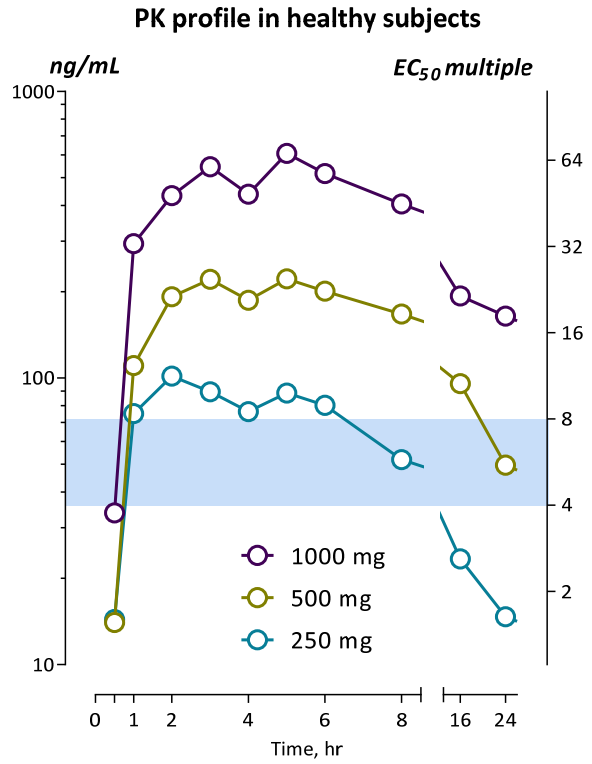
>300 patients enrolled with a total of >**100 patient years** on drug, 150 mg or 110 mg QD

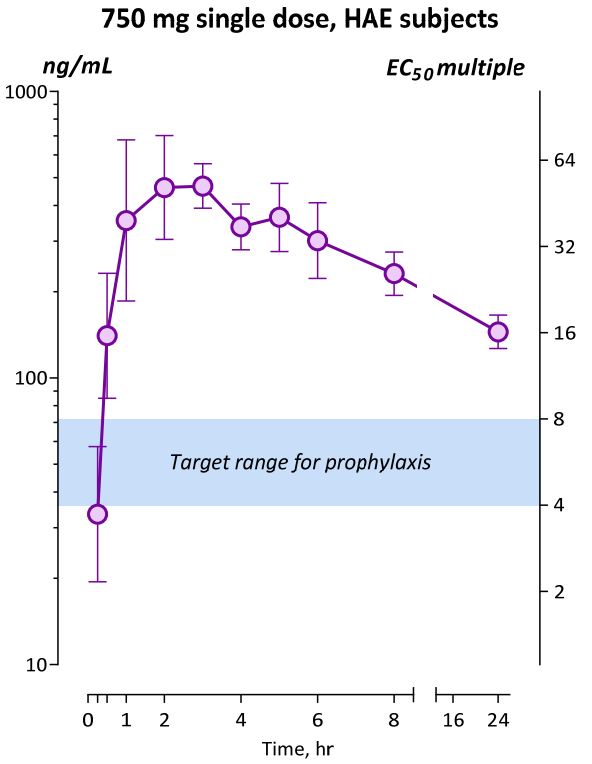
>80 patients on drug for more than **24 weeks**, with patients approaching one year on drug

24+ WEEKS

BioCryst HAE Acute Treatment Program (BCX7353)

PK Profiles of Single Oral Doses of BCX7353 Rapid Onset - Long Duration





Mathis A, et al *AAAAI*; *2018/11/01/*. 2018: S32.

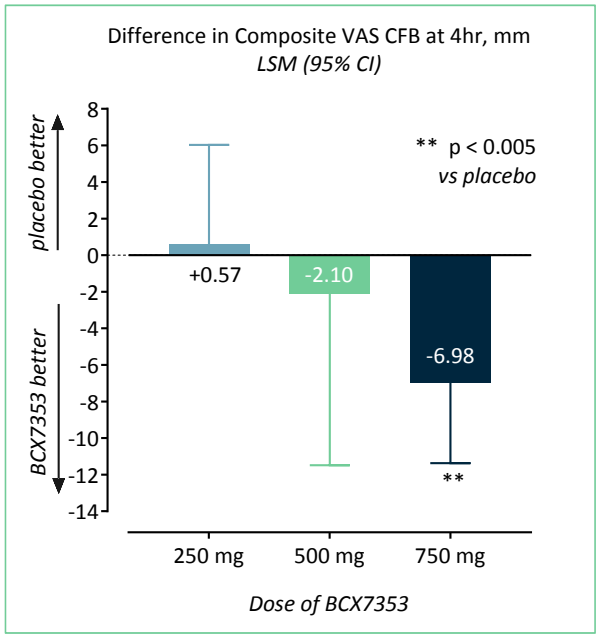
Data shown are means for healthy subjects and mean (SD) for HAE subjects

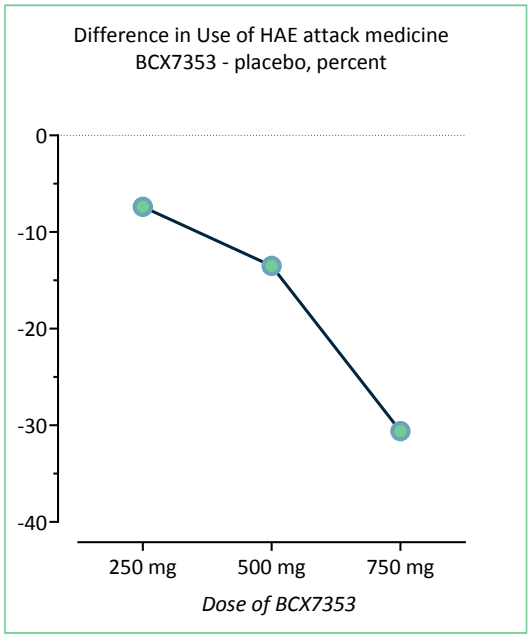
After a single oral dose of 750 mg BCX7353 in HAE subjects:

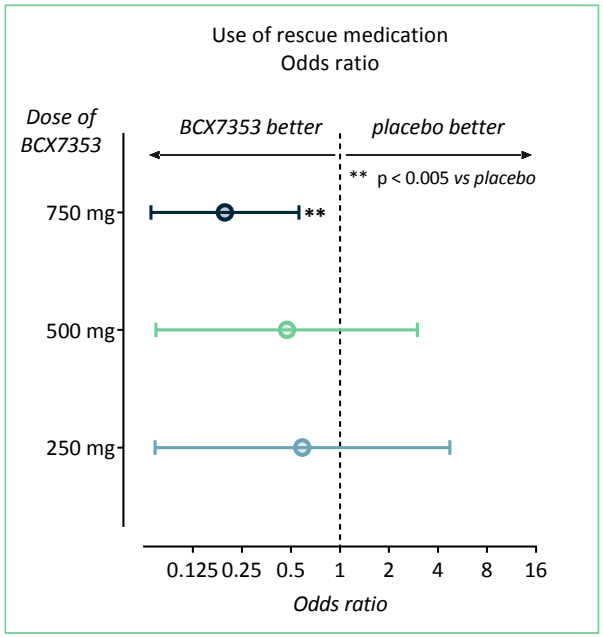
- Mean drug levels were approximately 16 x EC_{50} within 30 min, and remained at or above this level through at least 24 hours post-dose
- Drug concentrations exceeded 8 x EC_{50} in all subjects from 30 min to at least 24 hours post-dose

Mathis A, et al *AAAAI*; *2018/11/01/*. 2018: S32.

Robust Dose Response in ZENITH-1





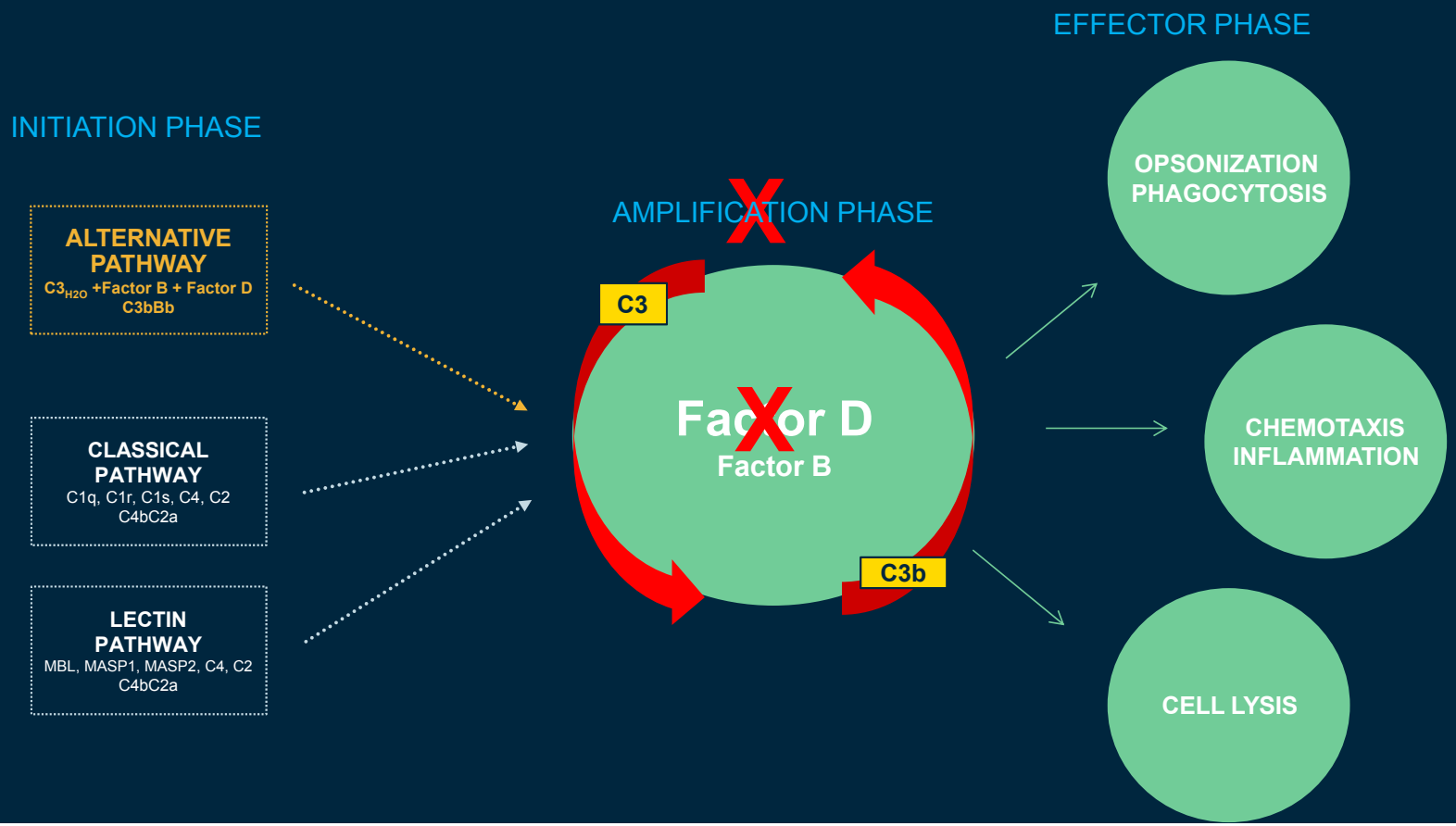


Longhurst, H. et al AAAAI 2019 San Francisco Poster #110

VAS=Visual Analog Scale CFB=Change from Baseline LSM=Least Square Mean

BioCryst Oral Factor D Inhibitor (BCX9930)

Targeting Factor D, the Rate Limiting Enzyme in the Alternative Pathway, Prevents Formation of Functional C3 Convertase Leading to Inhibition of Alternative Pathway Activity



Over $10 Billion Global Market Opportunity

Significant pipeline potential for a differentiated oral complement inhibitor

* SOLIRIS® (eculizumab) 2018 sales in paroxysmal nocturnal hemoglobinuria (PNH), atypical hemolytic uremic syndrome (aHUS), and generalized myasthenia gravis (gMG) reported 2/4/19
** Additional potential orphan indications for complement inhibitors include, but are not limited to, neuromyelitis optica (NMO), ANCA-associated vasculitis (AAV), C3 glomerulonephritis (C3G), IgA nephropathy (IgAN), warm autoimmune hemolytic anemia (wAIHA), focal segmental glomerulosclerosis (FSGS), and cold agglutinin disease (CAD)

BCX9930 a Potent and Selective Inhibitor of Factor D

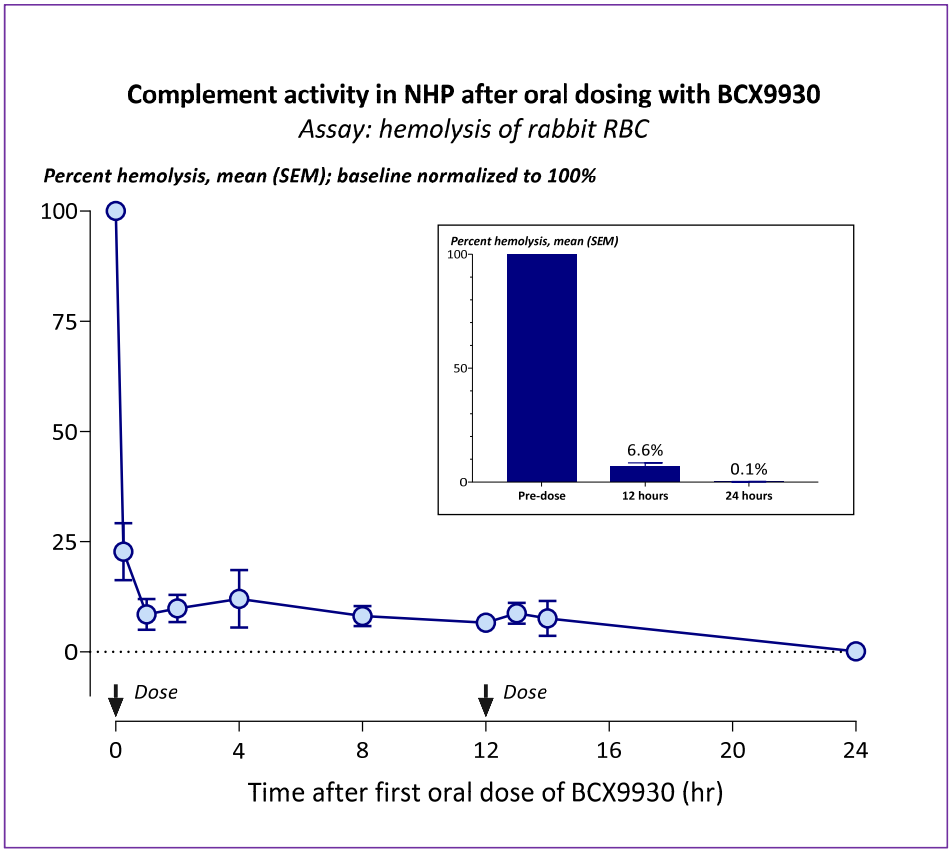
Potency Assays

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

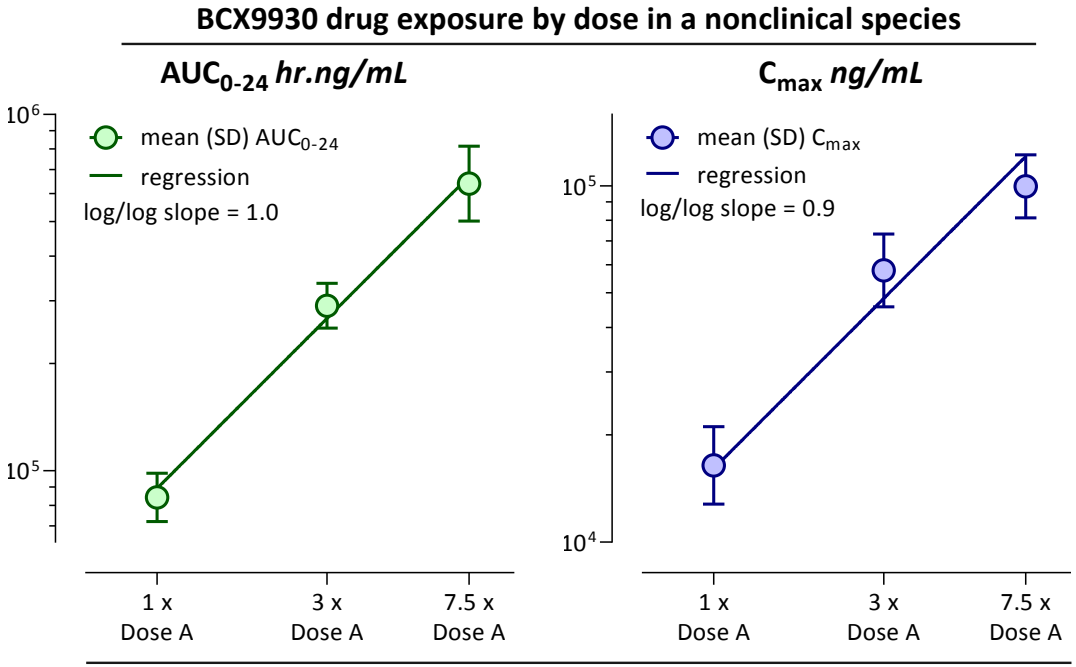
Serine Proteases	Selectivity Ratio relative to Factor D
Complement enzyme C1s	>60
Plasmin	≈ 200
Thrombin	>2000
Activated protein C	>2000
Tissue plasminogen activator	>2000
Trypsin	>2000
Factor Xa	>3000
Factor XIIa	>3000

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

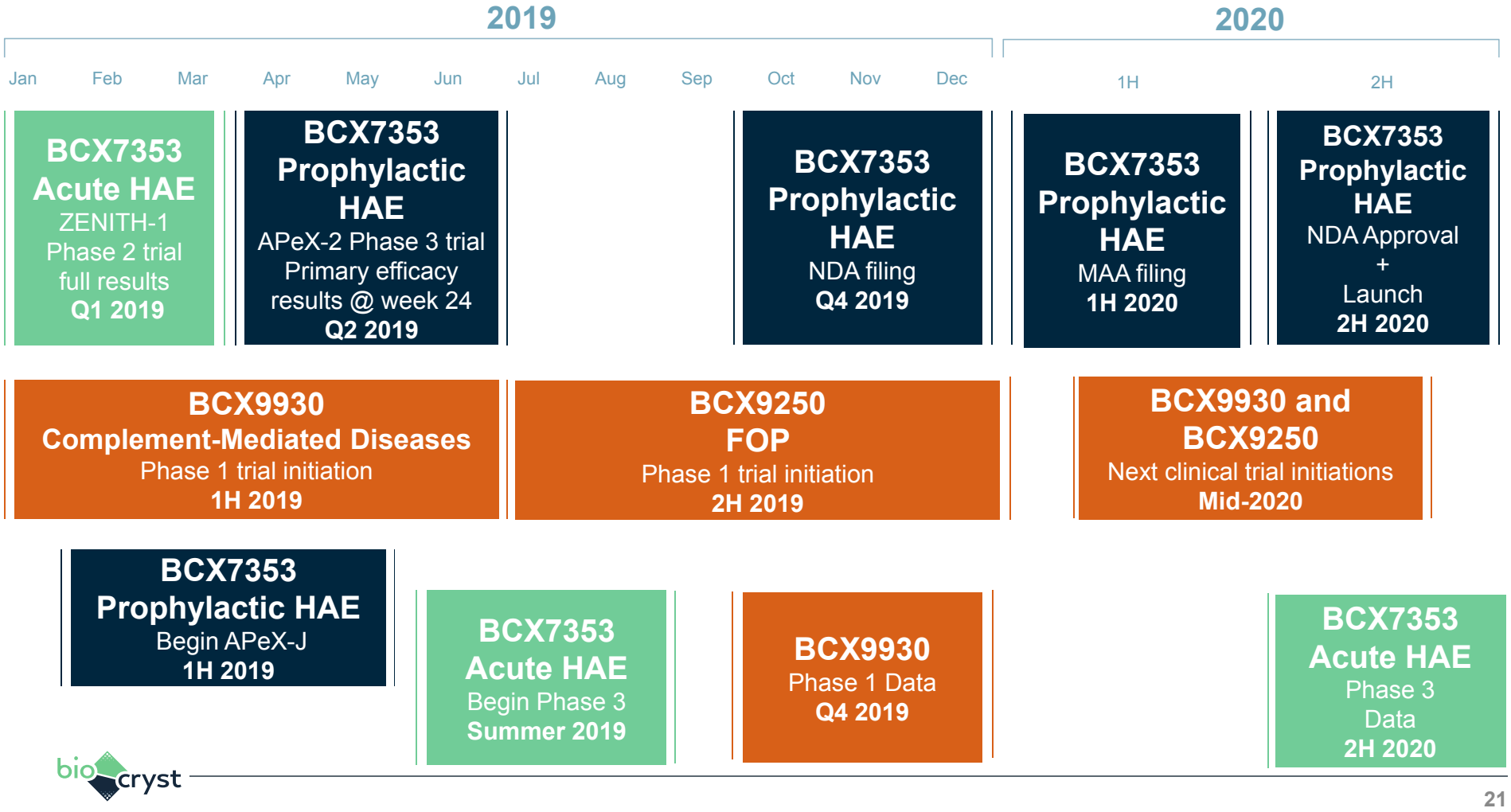
Cash Position & 2019 Guidance (in Millions)

Cash & investments at December 31, 2017	$159
Cash & investments at December 31, 2018[A]	**$128**
Senior Credit Facility [A]	$30
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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