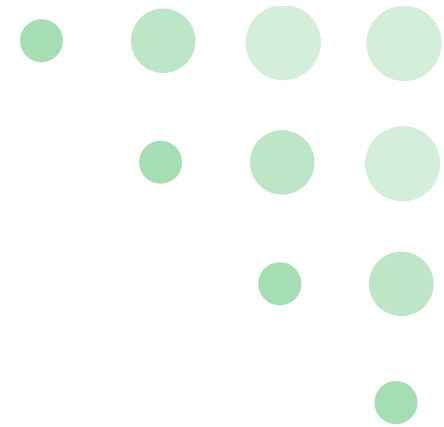


# 37<sup>th</sup> Annual J.P. Morgan Healthcare Conference

**Jon Stonehouse**  
Chief Executive Officer

January 9, 2019

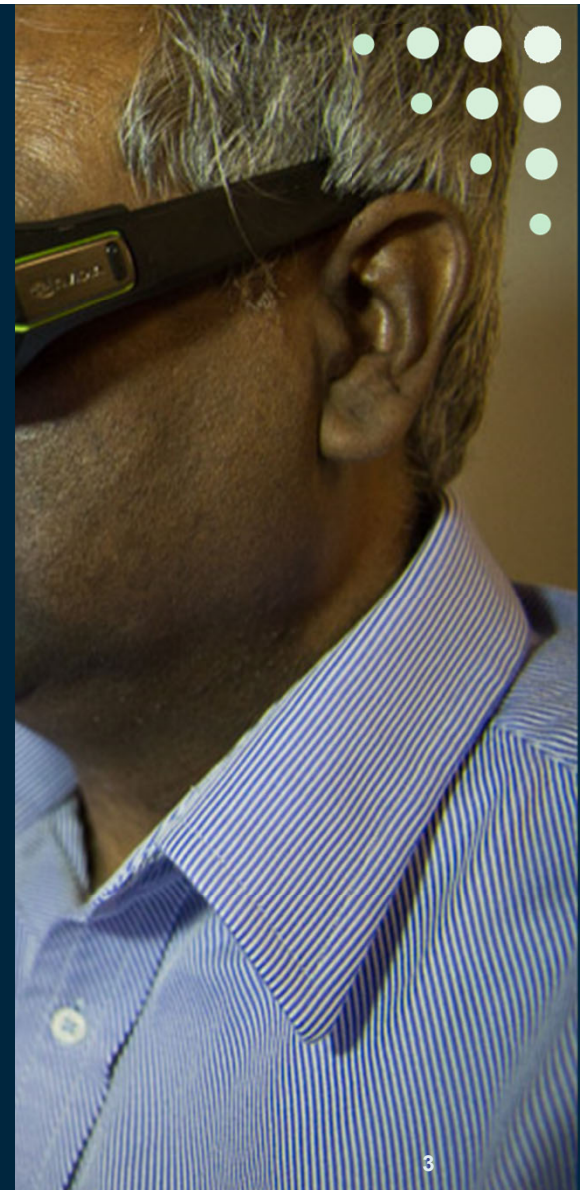


# Forward Looking Statements

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 results of the ZENITH-1 and APeX-1 trials may not be predictive of future results, including the results of the APeX-2, APeX-S, APeX-J trials and of the remaining cohorts of the ZENITH-1 trial; that developing BCX7353 for acute and prophylactic treatment may take longer or be more expensive than planned or may ultimately be unsuccessful; that producing commercial formulations 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 the 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.

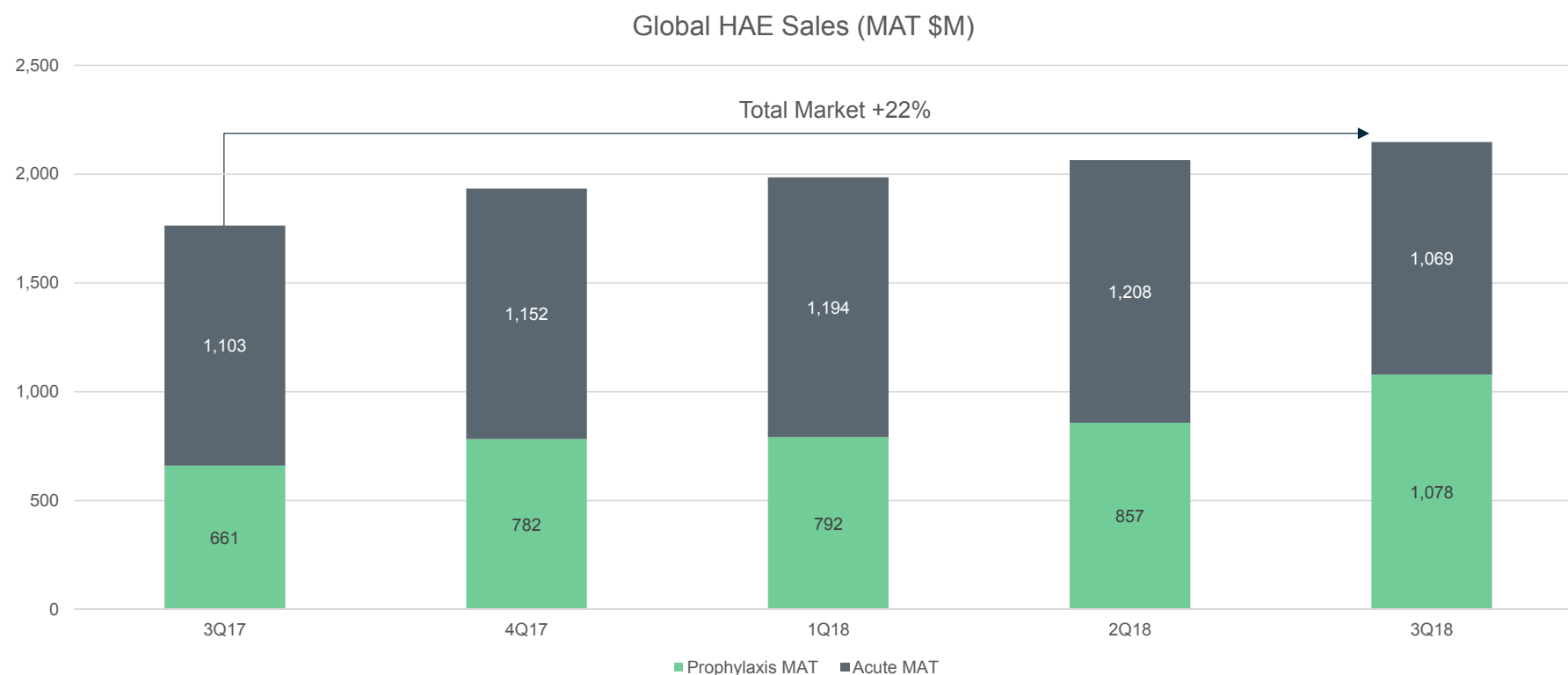
# Delivering Extraordinary Empowering Ordinary

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



# Annualized HAE Sales over \$2.1B Through 3Q18

Haegarda (3Q17) and Takhzyro (3Q18) launches driving prophylaxis past 50% of MAT sales



Sales based on actual reported sales for Shire products; actual reported sales for Pharming through 2Q18 and estimates through Q3; and estimates for CSL products based on publicly reported data and comments in 2017 and 2018.

# BCX7353 - A New Approach to Hereditary Angioedema Treatment



**Unpredictable,**  
debilitating, potentially  
life-threatening swelling  
attacks

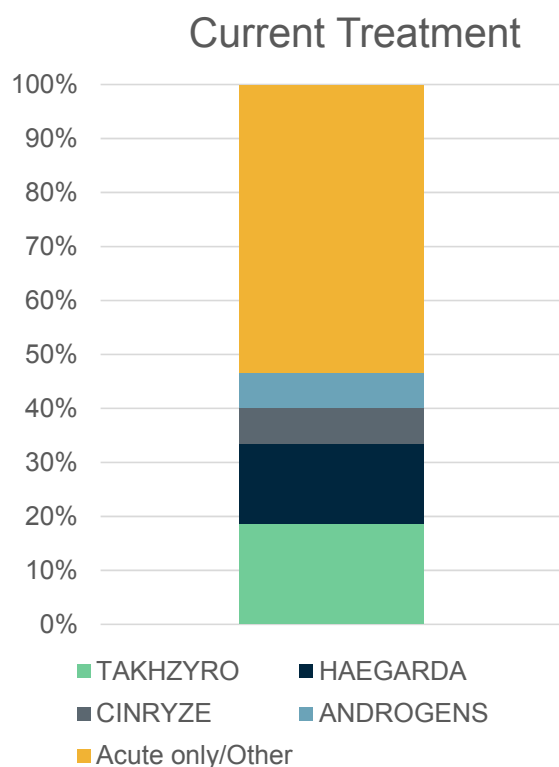
**1 in 50,000** people  
affected worldwide

**>\$2 Billion**  
global market opportunity

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

# 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)

*An oral prophylactic HAE medication **would fit my patients' lives** better than an injectable HAE medication*

**98%**  
agree

*If an oral prophylactic HAE medication becomes available, **I expect my HAE patients will try it***

**97%**  
agree

*When a patient **requests** a specific medication, **I prescribe** it if it is clinically appropriate*

**93%**  
agree

# BCX7353 Phase 2 APeX-1 Prophylaxis Proof of Concept

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

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

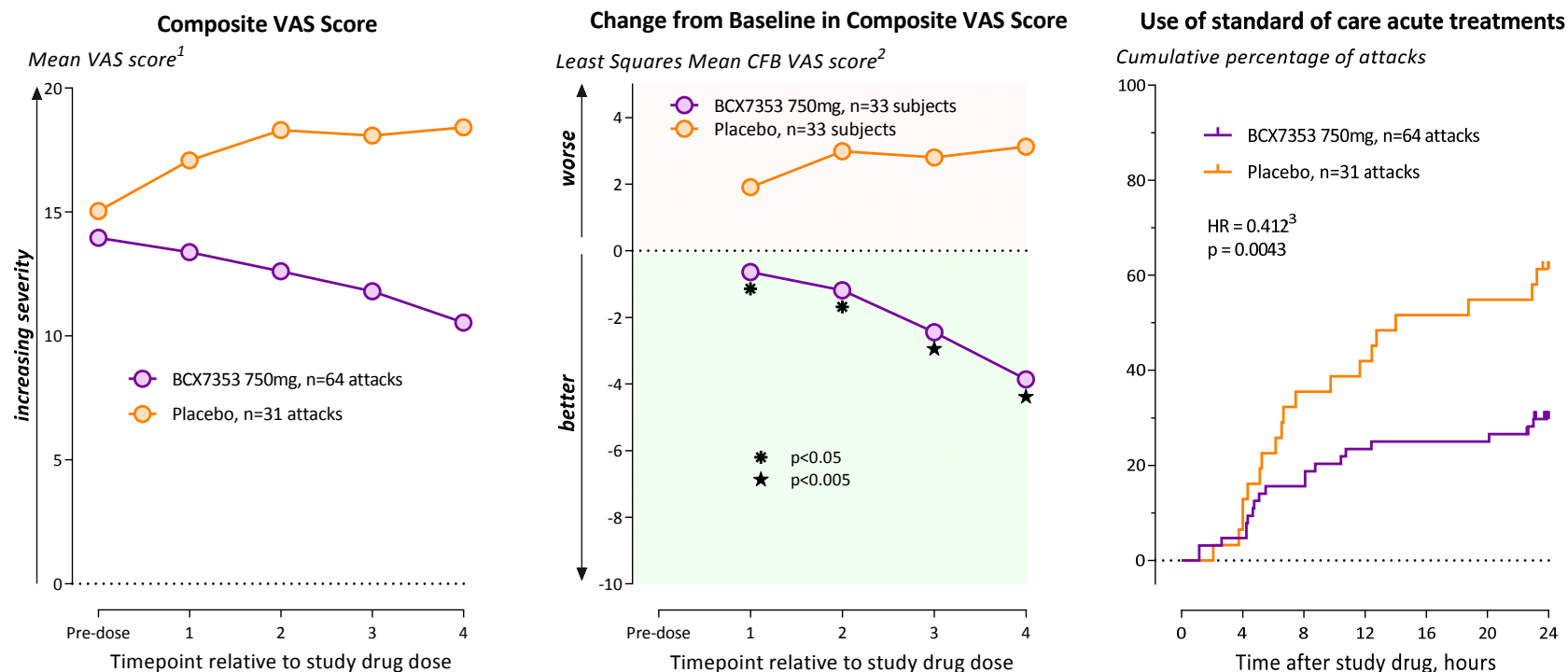


Final analysis



# BCX7353 Phase 2 Zenith-1 Acute Proof of Concept

## Rapid and Sustained Benefit from BCX7353



Values after standard of care treatments are excluded.

<sup>1</sup> The 3-symptom composite VAS was calculated as the average of three individual VAS scores of abdominal pain, cutaneous pain, and cutaneous swelling.

<sup>2</sup> 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.

<sup>3</sup> 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.

# In Proof of Concept Trials BCX7353 Generally Safe and Well Tolerated: APeX-1

Category	BCX7353				Placebo N = 22
	62.5 mg N = 7	125 mg N = 14	250 mg N = 14	350 mg N = 18	
Subjects with any TEAE <sup>1</sup> , n (%)	4 (57)	7 (50)	11 (79)	14 (78)	15 (68)
Subjects with any Serious AE, n (%)	0	0	1 (7) <sup>2</sup>	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) <sup>3</sup>	0
Drug-related, n (%)	0	0	0	2 (11) <sup>4,5</sup>	0

<sup>1</sup> TEAE- treatment-emergent adverse event.

<sup>2</sup> 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.

<sup>3</sup> Pre-existing liver disorder (improved from baseline, but persisting). Previously reported in 1<sup>st</sup> interim analysis.

<sup>4</sup> 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 1<sup>st</sup> interim analysis.

<sup>5</sup> n=1 Vomiting/abdominal cramps. Previously reported in 2<sup>nd</sup> interim analysis.

# In Proof of Concept Trials BCX7353 Generally Safe and Well Tolerated: ZENITH-1, Part 1

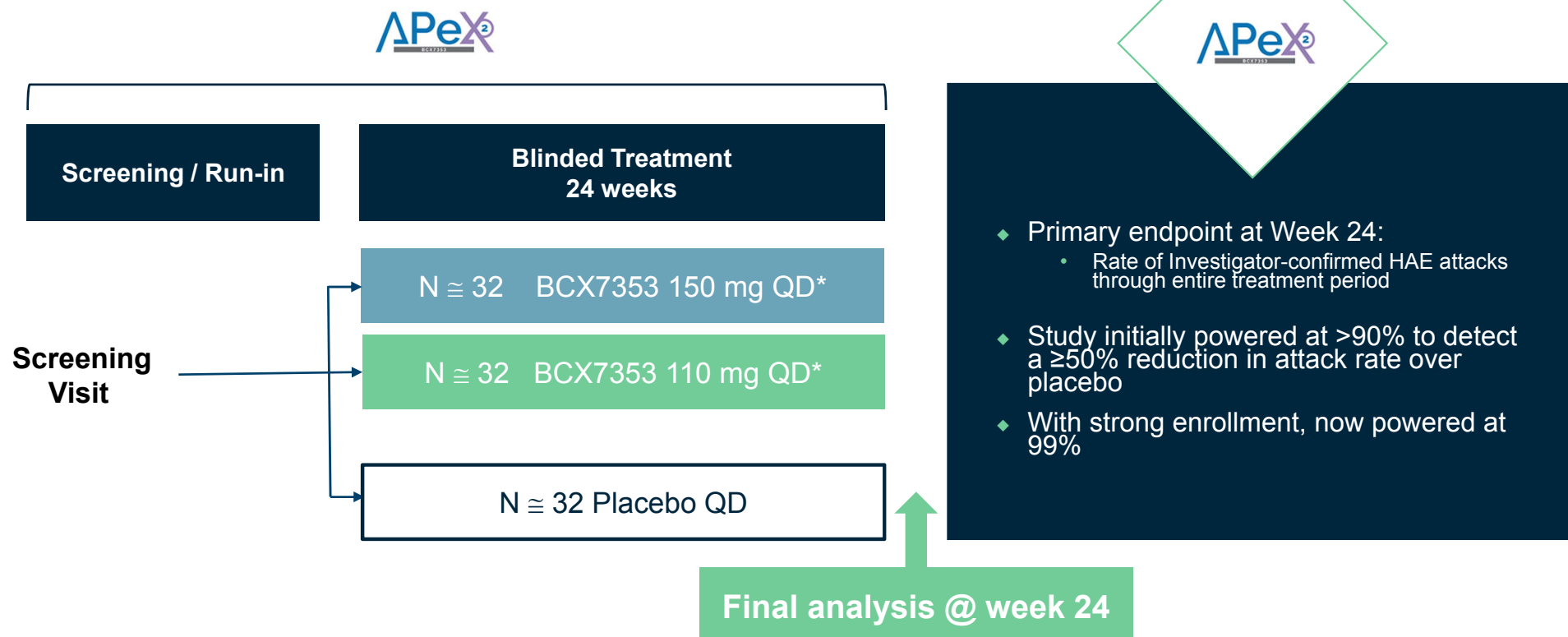
Category	BCX7353 (750 mg)	Placebo
Number of subjects	33	31
Number of attacks treated*	64	31
Number of attacks with a reported treatment-emergent adverse events (TEAE)	16 ( 25.0%)	7 ( 22.6%)
Number of attacks with a serious TEAEs	0	1 ( 3.2%)
Number of attacks with a drug-related TEAEs as assessed by investigator	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		
<i>Nasopharyngitis</i>	4 (6.3%)	1 (3.2%)
<i>Diarrhea</i>	3 (4.7%)	0
<i>Headache</i>	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.

# APeX-2: Phase 3 Trial Fully Enrolled



\*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**

# Regulatory Agency Status for BCX7353



- Orphan Drug Designation
- EOP2
- Fast Track Designation



EUROPEAN MEDICINES AGENCY  
SCIENCE MEDICINES HEALTH

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

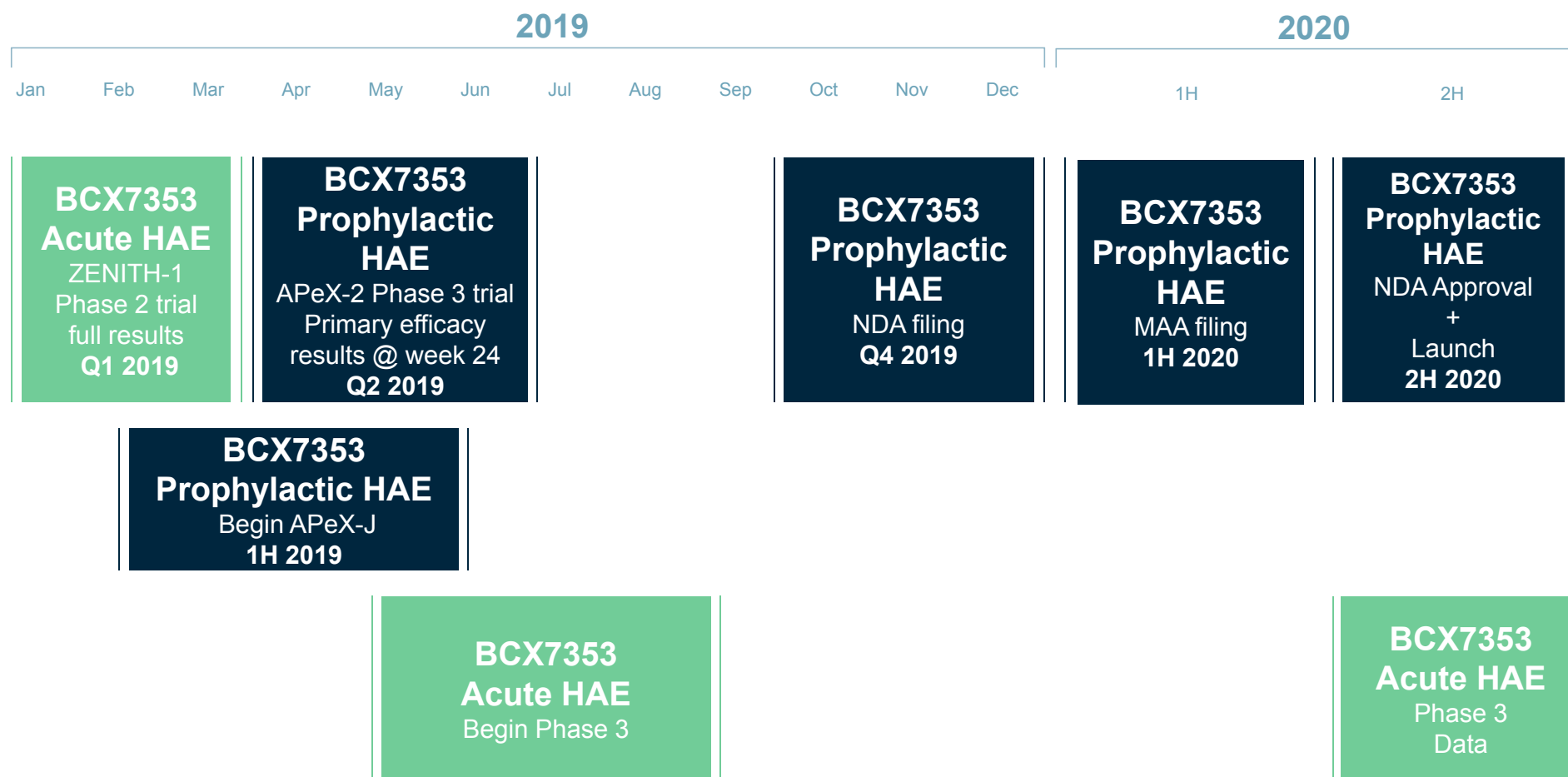


- UK Promising Innovative Medicine (PIM)



- Orphan Drug Designation
- Formal Consultation Process (EOP2 equivalent)
- Sakigake Designation

# HAE: Value Creating Milestones Leading to 1st Launch



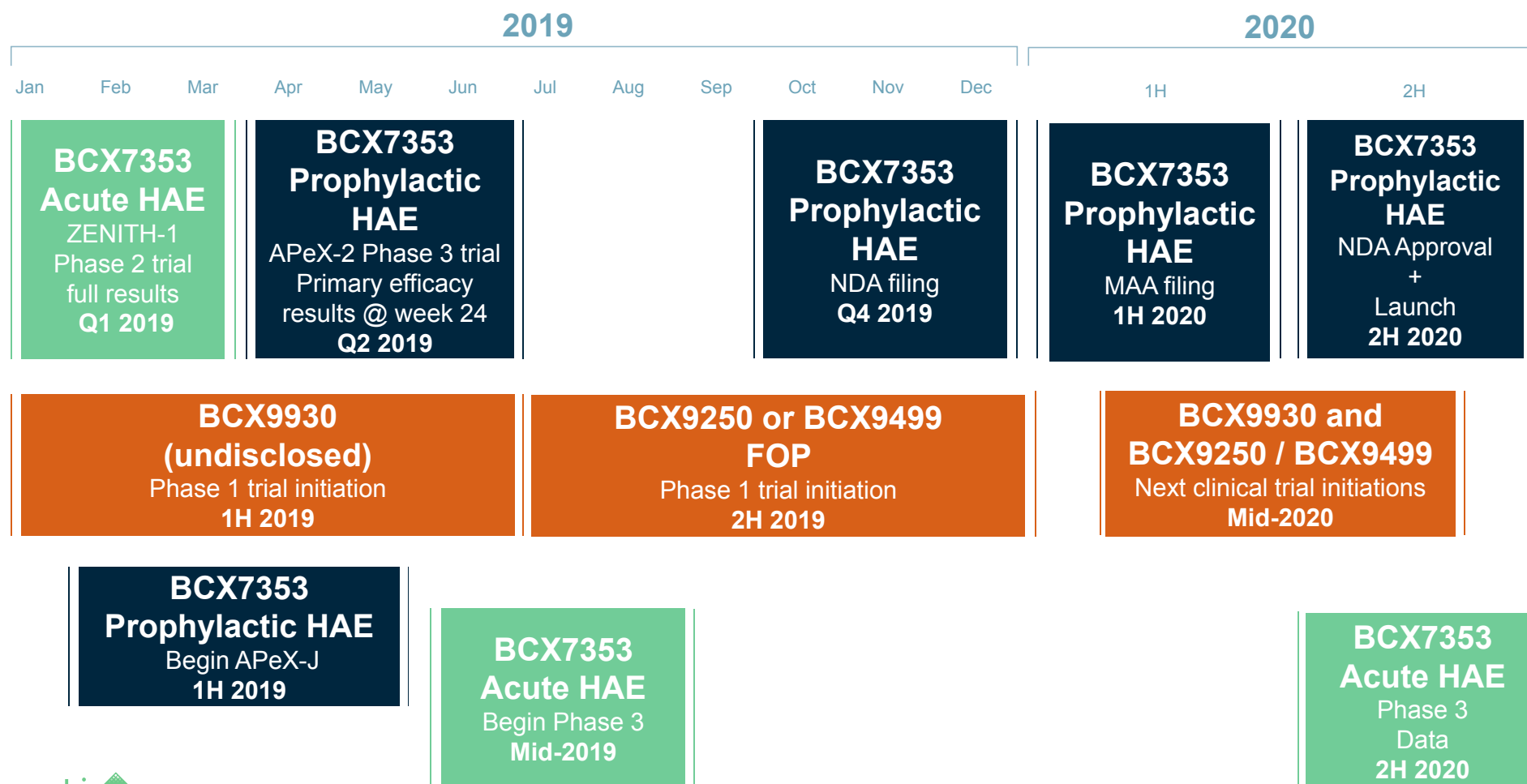
# BioCryst's Robust Pipeline

	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 (undisclosed indication)							
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



# Many Anticipated Milestones in 2019 - 2020



## Cash Position & 2018 Guidance (in Millions)

Cash & investments at December 31, 2017	\$159
Cash & investments at September 30, 2018	\$151
Senior Credit Facility <sup>A</sup>	\$30
FY 2018 GUIDANCE	
Operating cash utilization	\$85 – 105
Operating expenses <sup>B</sup>	\$90 – 110

<sup>A</sup> - Credit Facility was enhanced in July 2018.

<sup>B</sup> - Excludes equity-based compensation.



# 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 7353—Into the clinic next year
  - 9930 (undisclosed indication)
  - FOP
- Well capitalized
- Next 18 months: Multiple value creating milestones

# 37<sup>th</sup> Annual J.P. Morgan Healthcare Conference

**Jon Stonehouse**  
Chief Executive Officer

January 9, 2019

