

# Jefferies 2019 Healthcare Conference

**Jon Stonehouse**  
Chief Executive Officer

**Dr. Bill Sheridan**  
Chief Medical Officer

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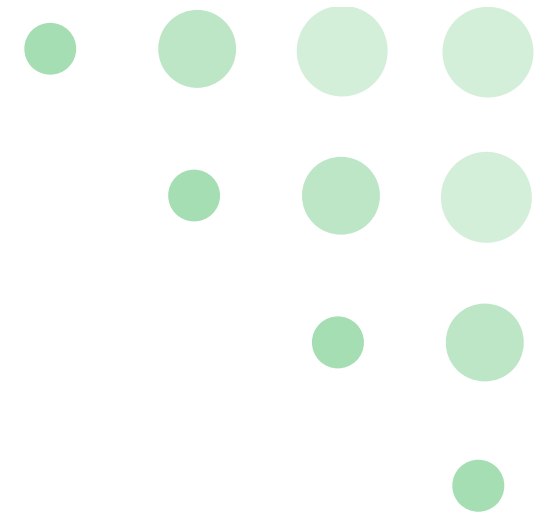
# Forward-Looking Statements

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>

A man with grey hair and a mustache, wearing a blue and white striped shirt and black AR glasses, is looking at a computer monitor. The monitor displays a 3D molecular model with blue and red components. The background is dark, and there are several white dots in the top right corner.

# Delivering extraordinary Empowering ordinary

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



# BioCryst HAE Prophylactic Program (BCX7353)



# Responder Analyses for BCX7353 150 mg

APeX-2 Primary Endpoint (150mg): 44% overall reduction in HAE attack rate vs placebo (p<0.001)

Response outcome	Placebo n=39*	BCX7353 150 mg n=40		
	Percent	Percent	Odds ratio	P value ‡
≥ 50% reduction	25.0	57.5	3.9	0.005
≥ 70% reduction	15.0	50.0	5.6	0.002
≥ 90% reduction	7.5	22.5	3.6	0.073

\* One of 40 placebo subjects did not receive blinded study drug and did not contribute attack rate information after randomization  
‡ Logistic regression model

# Overall Safety Summary

Treatment-emergent (TE) Adverse Events (AEs) or Discontinuations (DCs) due to TEAEs	BCX7353 110 mg	BCX7353 150 mg	Placebo
	N = 41	N = 40	N = 39*
Drug-Related TE Serious AEs	0	0	0
Most common Drug-Related AEs			
Nausea	4 (9.8%)	3 (7.5%)	6 (15.4%)
Dyspepsia	4 (9.8%)	3 (7.5%)	2 (5.1%)
Diarrhea	3 (7.3%)	4 (10.0%)	0
DCs due to TEAEs	3 (7.3%)	1 (2.5%)	1 (2.6%)
DCs due to Abdominal-Related GI TEAEs	1 (2.4%)	0	0
DCs due to Abnormal Liver Function Test	0	1 (2.5%)	0

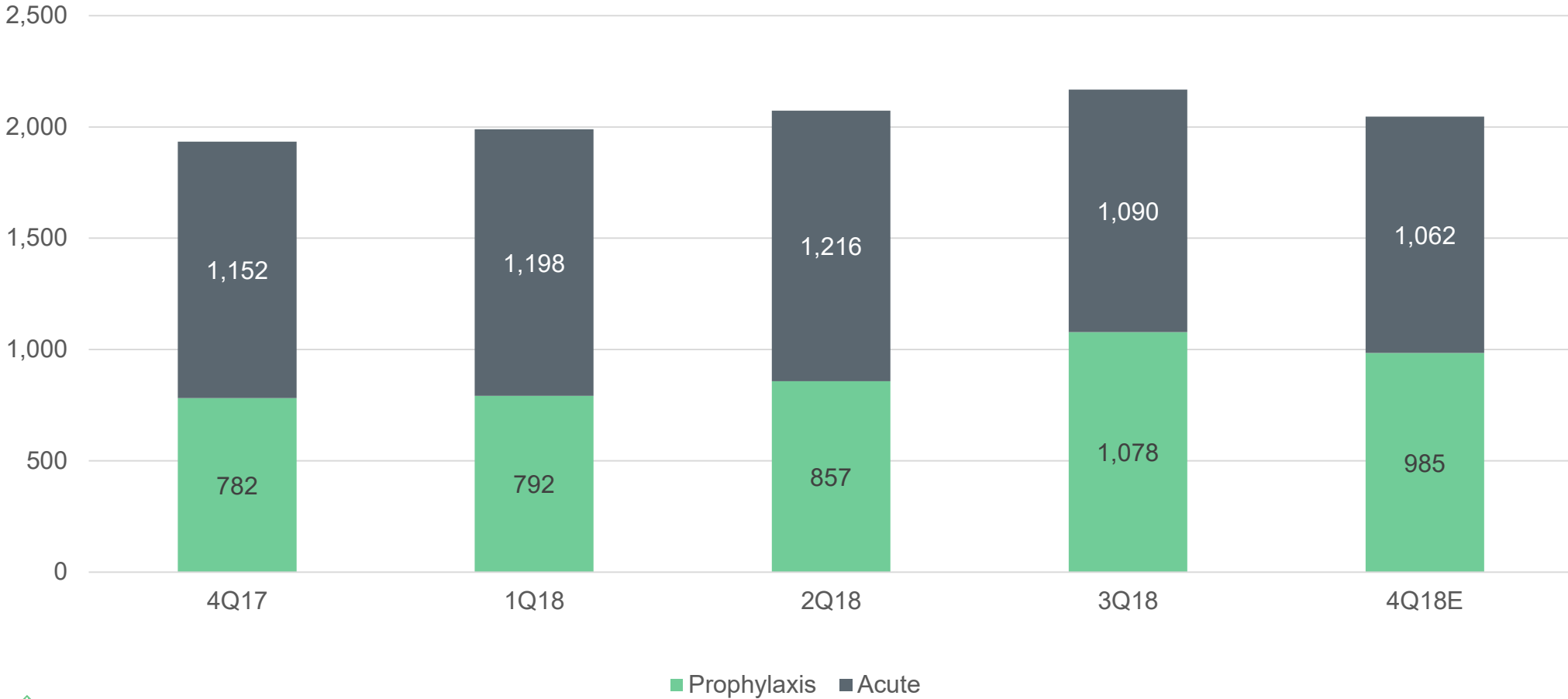
\* One of 40 placebo subjects did not receive blinded study drug and did not contribute attack rate information after randomization

# Global HAE Sales Exceeded \$2.0 Billion in 2018



Prophylactic sales were 48% of total compared to 40% in 2017

Moving 12-month HAE Sales (\$M)



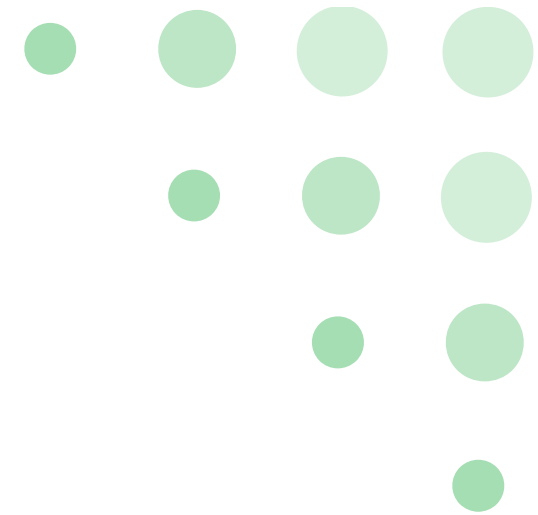


# Key Conclusions from APeX-2 Topline Data



- ◆ **BCX7353 is approvable**
  - Clinically meaningful attack rate reduction ( $p < 0.001$ ) on primary endpoint for both doses
  - Excellent safety/tolerability profile
  - Consistent dose response across endpoints
  - New treatment option (oral) meeting significant unmet medical need
- ◆ **Significant reduction in attack rate for many patients will drive high trial usage rates**
  - 23% of patients had  $\geq 90\%$  reduction in attack rate
  - 50% of patients had  $\geq 70\%$  reduction in attack rate
- ◆ **BioCryst will lower barriers to enable/accelerate first-line access in U.S/EU/RoW**
- ◆ **Tremendous excitement about data from physicians and patients**

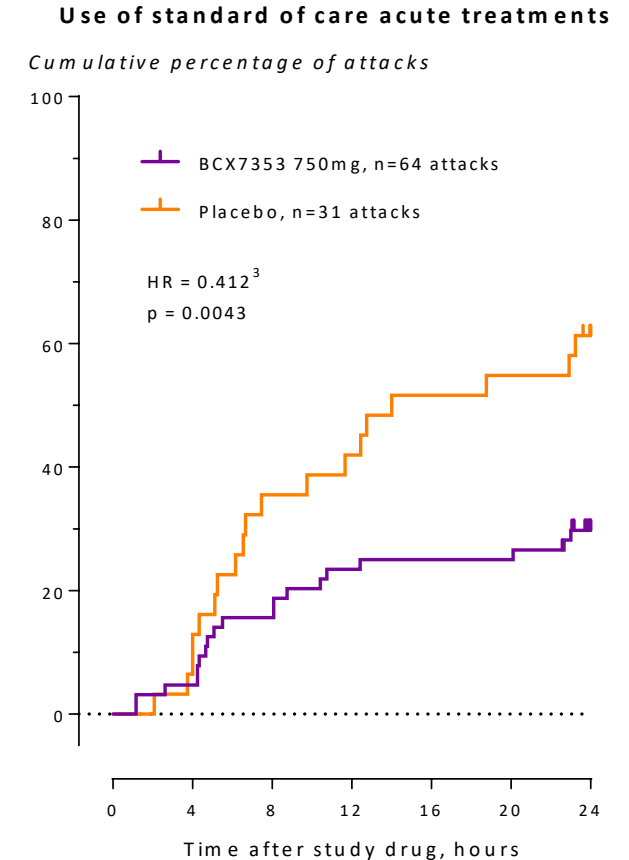
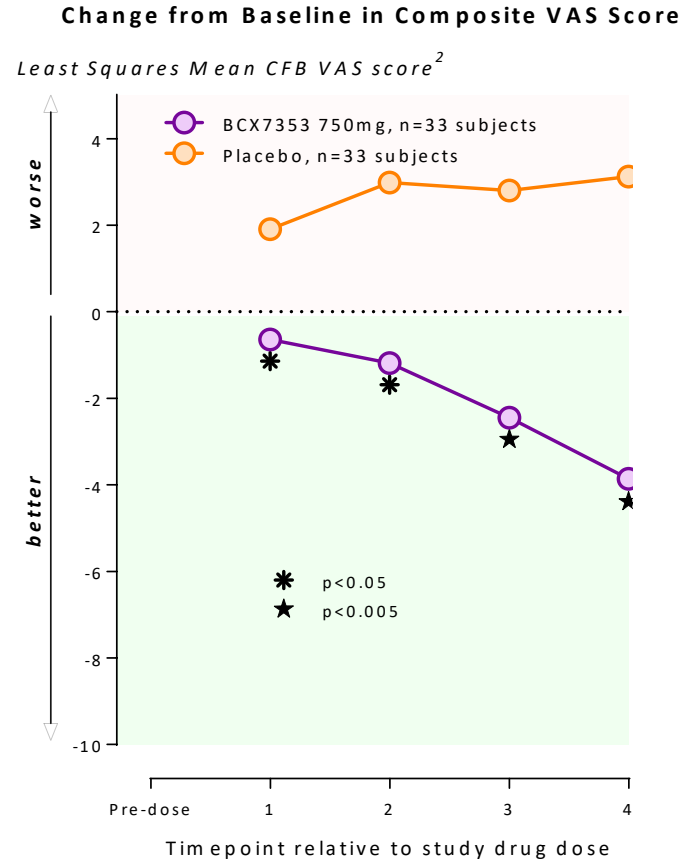
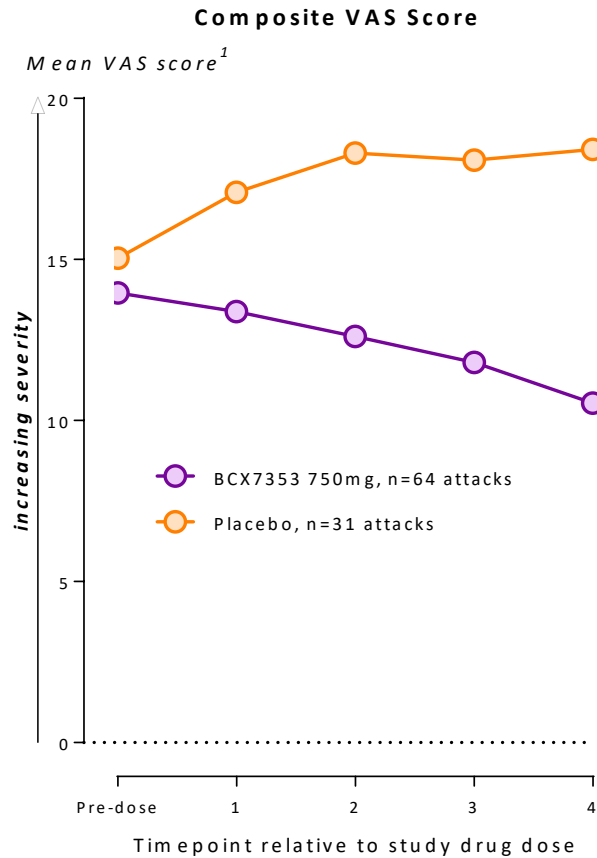




# BioCryst HAE Acute Treatment Program (BCX7353)



# 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.

# 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.

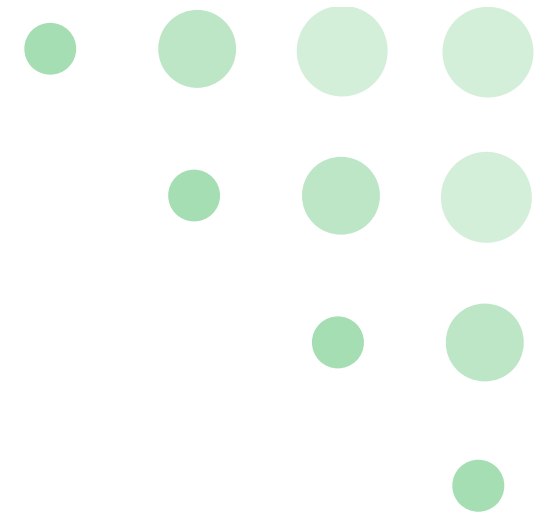
Δ Grade 3 serious TEAE of Ankle fracture

‡ 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.

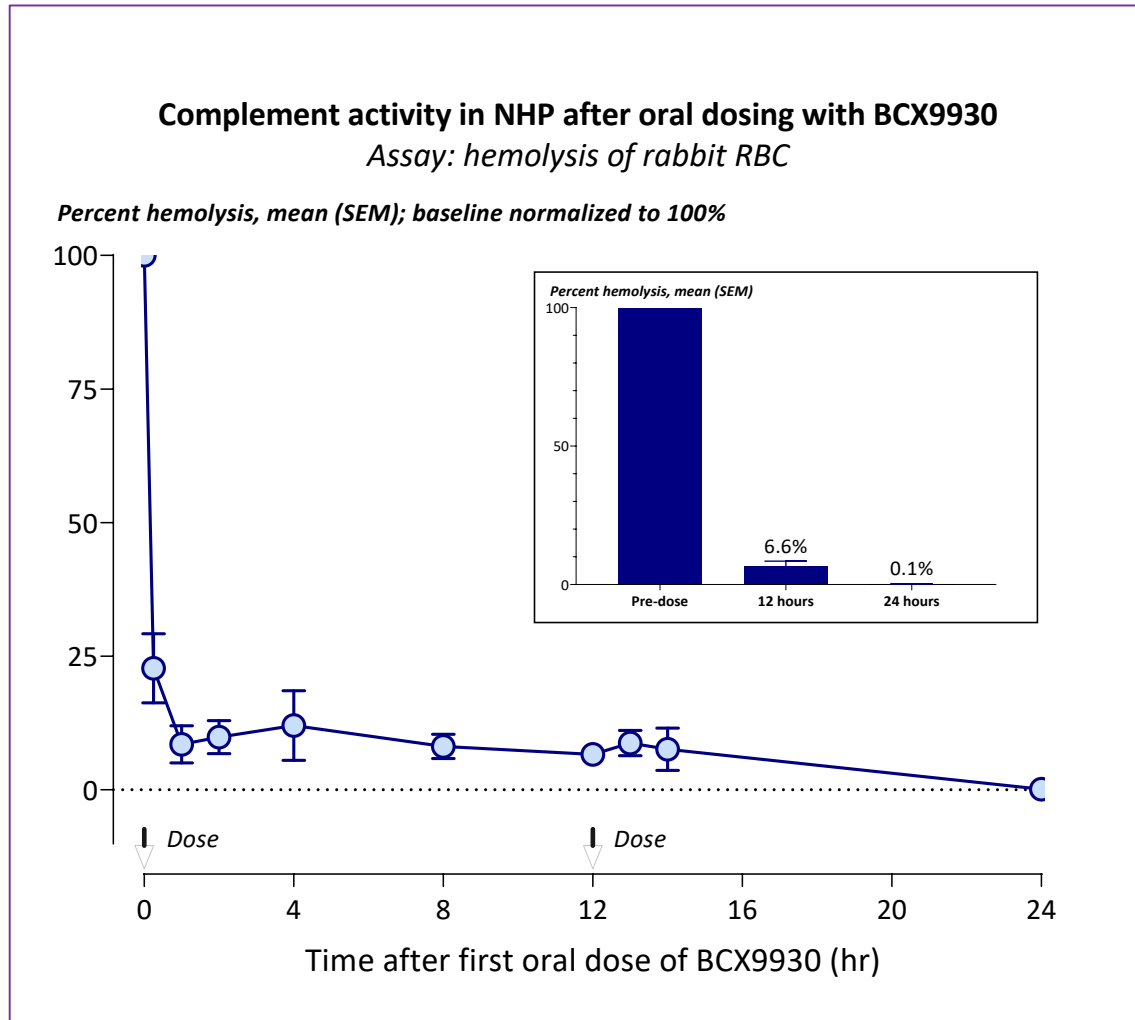
€ Discontinuation on BCX7353 occurred in a subject who experienced moderate vomiting and nausea.



# BioCryst Oral Factor D Inhibitor (BCX9930)



# BCX9930 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

# Cash into position & 2019 guidance (in millions)



Cash & investments at December 31, 2018	\$128
Cash & investments at March 31, 2019	\$122
Senior Credit Facility <sup>A</sup>	\$50
<b>FY 2019 GUIDANCE</b>	
Operating cash utilization	\$105 – 130
Operating expenses <sup>B</sup>	\$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.

# BioCryst's Robust Pipeline



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

\*Licensed to Seqirus, Shionogi and Green Cross



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