Needham 19th Annual Healthcare Conference

Jon Stonehouse
Chief Executive Officer

Dr. Bill Sheridan
Chief Medical Officer

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Forward-Looking Statements

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BioCryst in 2020: Approvals, Launches and Data

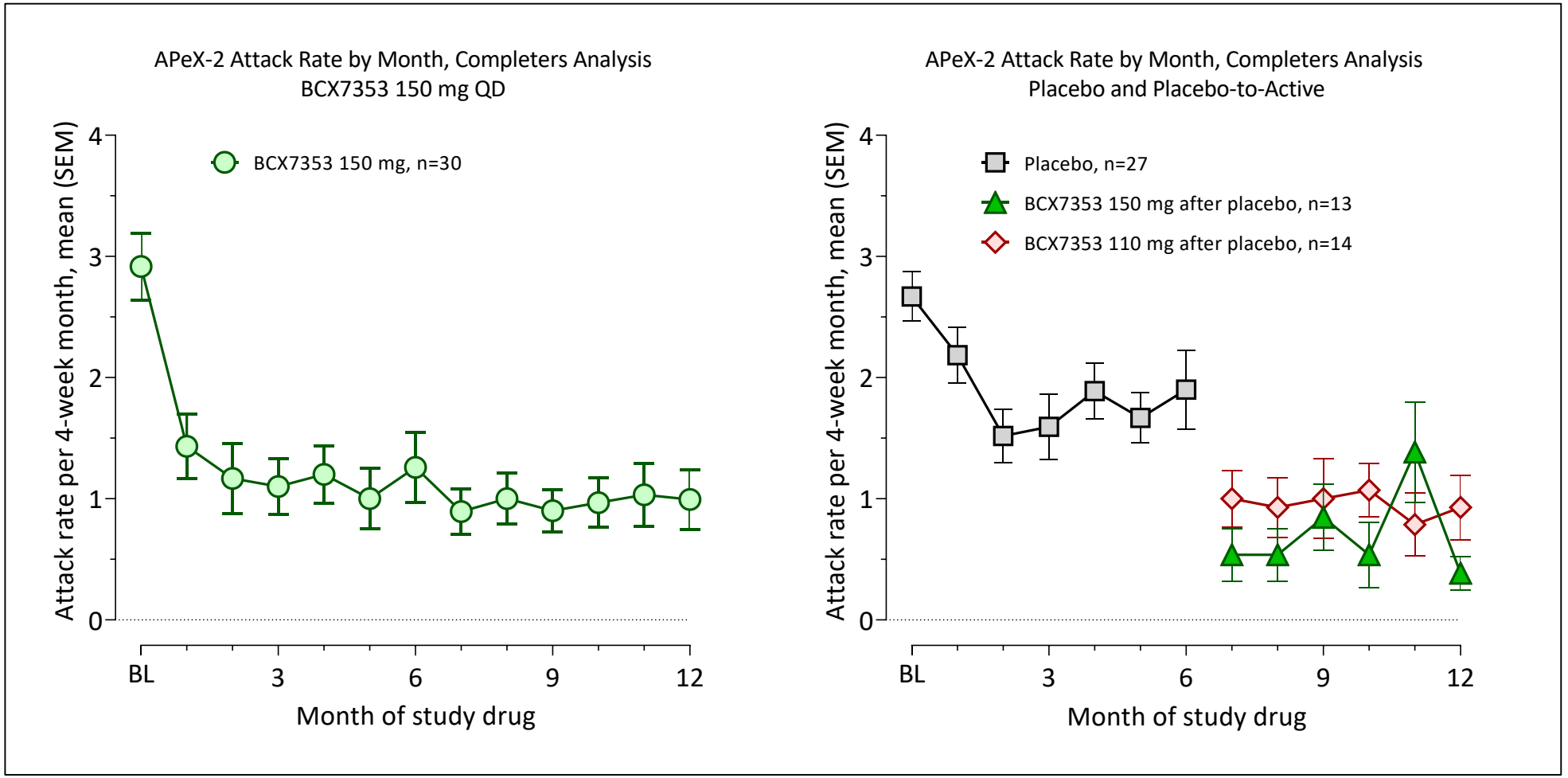
2019 Accomplishments

- ✓ Submitted NDA to FDA for berotralstat
- ✓ Initiated oral Factor D Phase 1 trial for complement-mediated diseases
- ✓ Initiated oral ALK2 inhibitor Phase 1 study for development in FOP
- ✓ Added ~$100M in capital during 4Q 2019

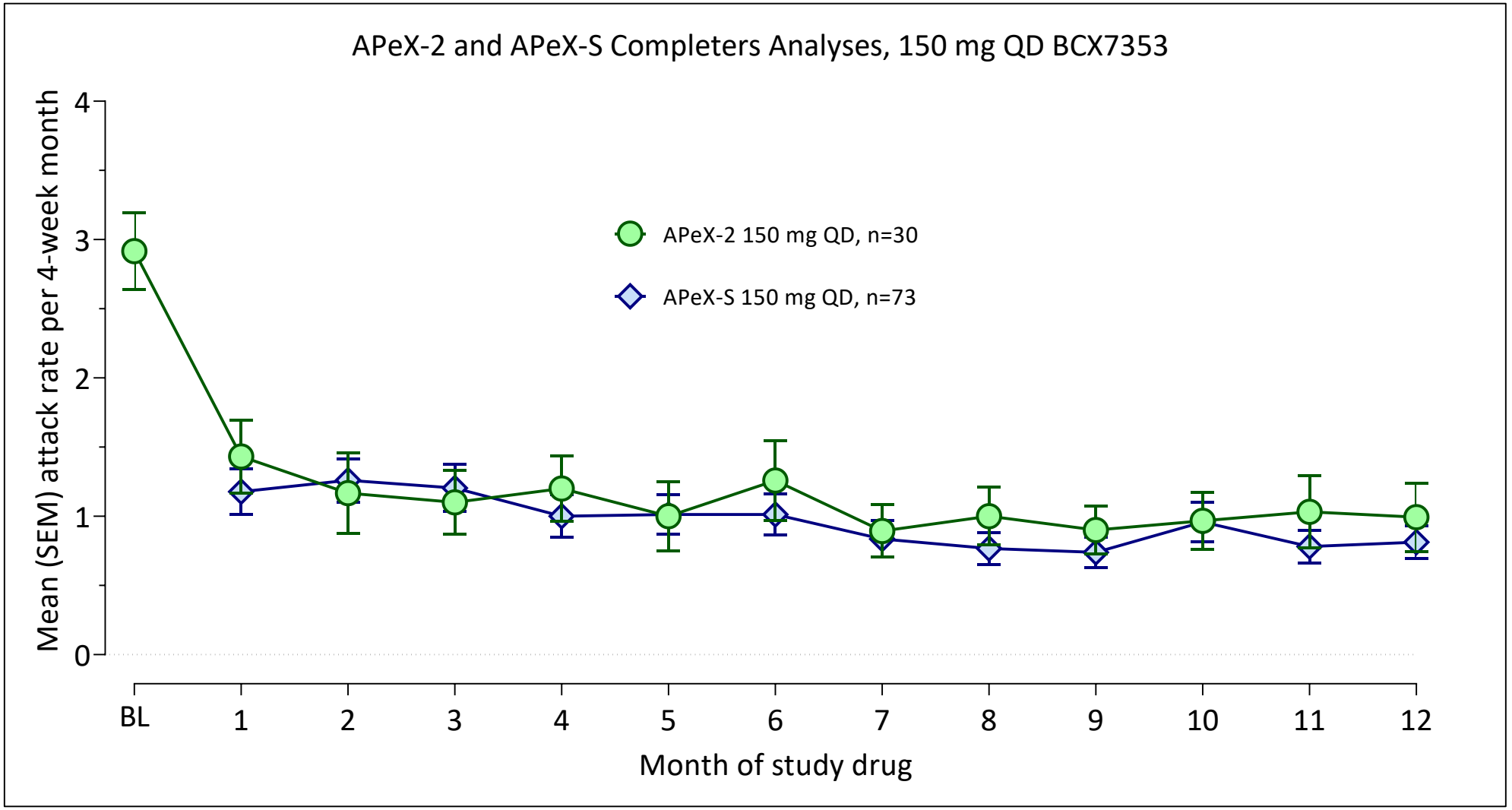
2020 Priorities

1. **Obtain berotralstat approvals in U.S. + Japan and submit MAA to EMA**
2. **Prepare commercial infrastructure for successful launches in the US & EU (+ support Torii in Japan)**
3. **Achieve proof of concept for oral Factor-D inhibitor in PNH patients**
4. **Continue advancing rare disease portfolio via in-house R&D or out-licensing partnerships**

Rapid and Sustained HAE Attack Rate Reduction: Analyses for APeX-2: 150 mg and Placebo 48-week Completers

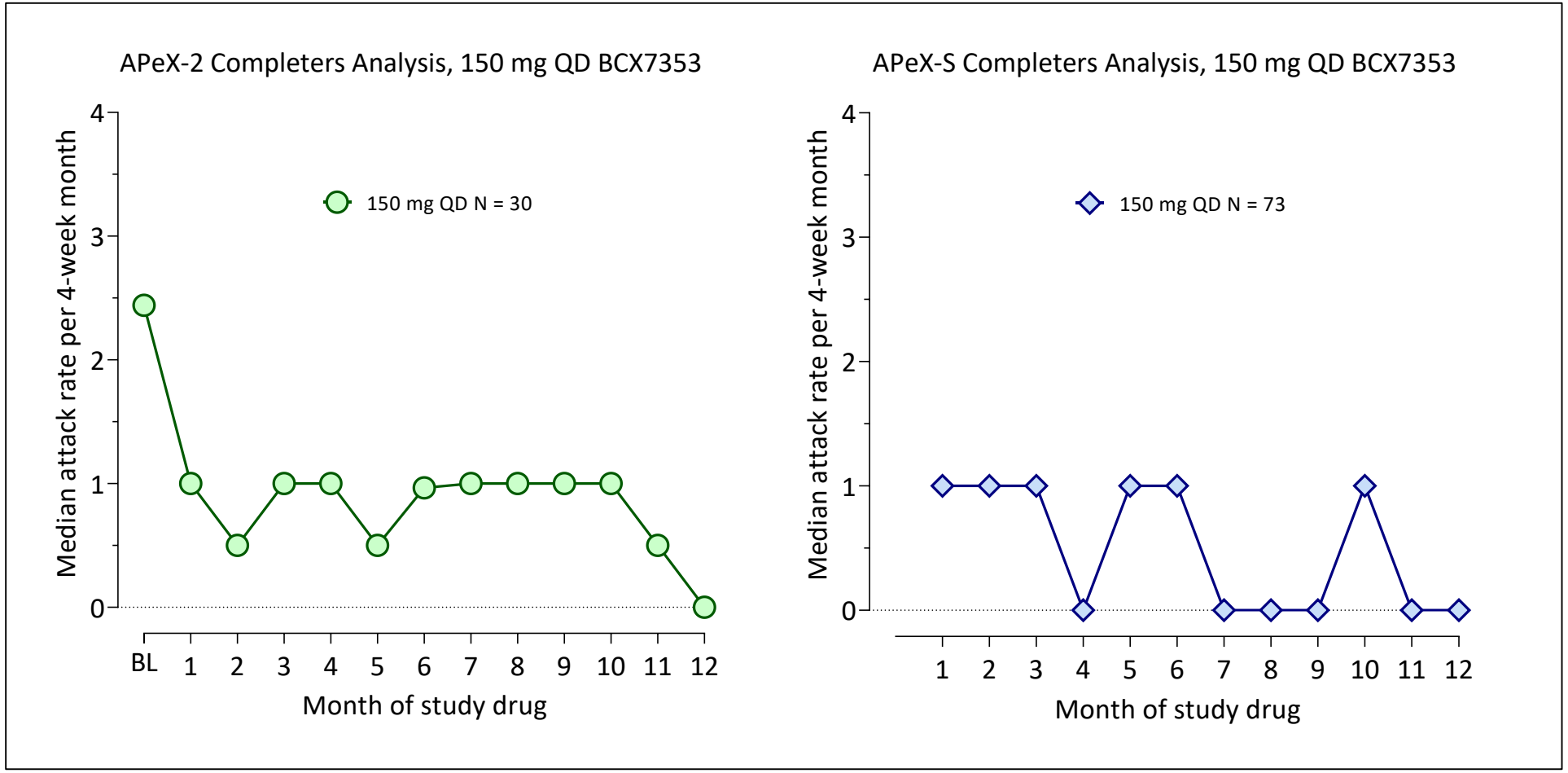


Consistent Mean Attack Rates in APeX-2 and APeX-S



Median Attack Rates in 48-week Completers:

Zero Attacks per Month in 6 of 12 Months in APeX-S



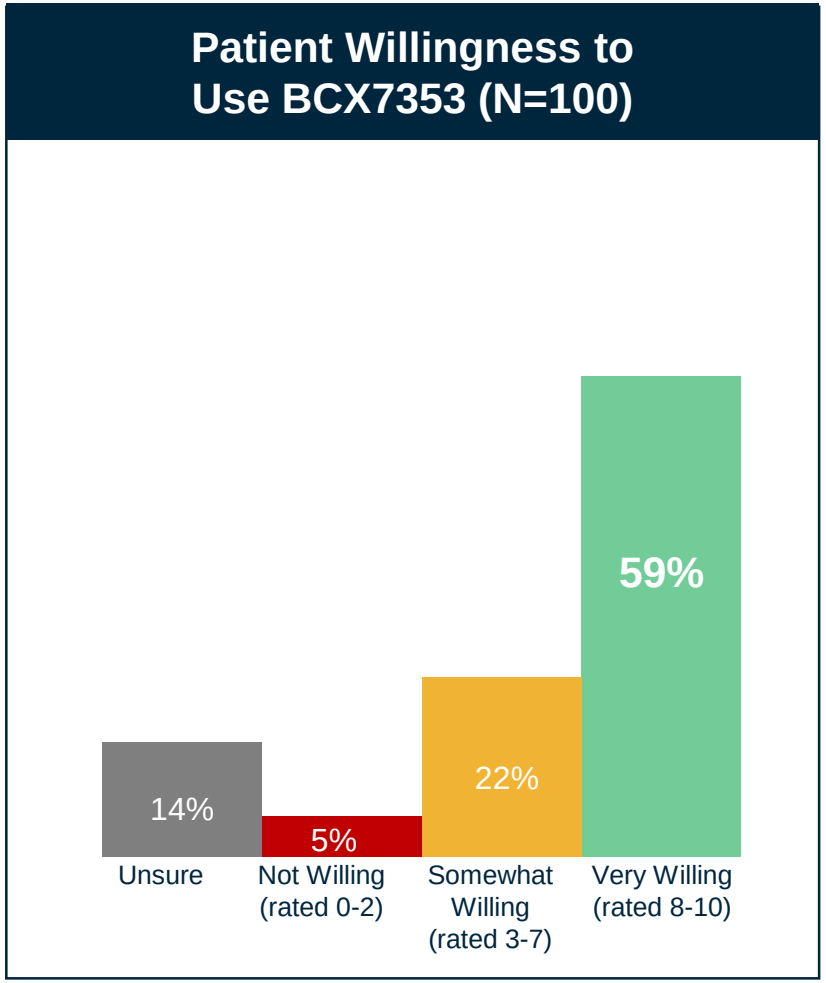
Safety and Tolerability Confirmed in Integrated 48-week Analysis

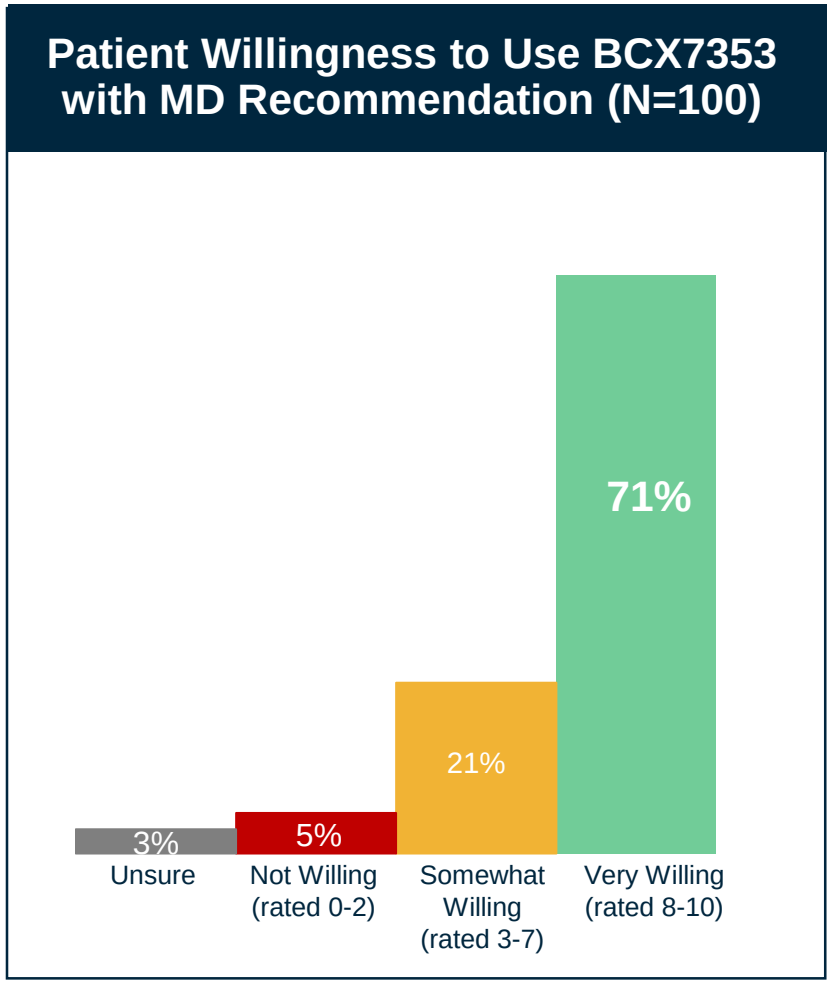
Integrated Safety Summary – APeX-2 and APeX-S	BCX7353 110 mg	BCX7353 150 mg	Placebo
Subjects enrolled and dosed [Safety Population]	***N = 158***	***N = 184***	***N = 39***
Subject Incidence of SAEs or Discontinuations due to AEs			
Drug-Related Serious AEs	2 (1.3%) [1, 2]	1 (0.5%) [3]	0
AEs Leading to Discontinuation of Study Drug			
Abdominal GI AEs [4]	4 (2.5%)	7 (3.8%)	0
Abnormal Liver Function Test	3 (1.9%)	6 (3.3%)	0
Other AEs	4 (2.5%) [5]	5 (2.7%)	1 (2.6%)
Subject Incidence of Most Common GI Abdominal AEs Reported as Drug-Related [6]			
Gastrointestinal Disorders System Organ Class	62 (39.2%)	65 (35.3%)	11 (28.2%)
Nausea	10 (6.3%)	15 (8.2%)	6 (15.4%)
Abdominal pain	14 (8.9%)	16 (8.7%)	0
Diarrhea	10 (6.3%)	15 (8.2%)	0
Flatulence	4 (2.5%)	11 (6.0%)	1 (2.6%)
Abdominal pain upper	9 (5.7%)	7 (3.8%)	1 (2.6%)
Dyspepsia	8 (5.1%)	10 (5.4%)	2 (5.1%)
Abdominal discomfort	7 (4.4%)	6 (3.3%)	2 (5.1%)
Abdominal distension	5 (3.2%)	8 (4.3%)	2 (5.1%)
Vomiting	4 (2.5%)	7 (3.8%)	0

1: Gastroenteritis and hepatic enzyme increased in the same subject, events resolved after stopping study drug (ApeX-S)
2: Abdominal pain, event resolved after interrupting study drug (ApeX-S)
3: LFT abnormal, event resolved after stopping study drug (ApeX-S)
4: GI abdominal-related AEs were any AEs with a PT within the MedDRA 19.1 hierarchy under the high level group terms of GI signs and symptoms or GI motility and defecation conditions
5: One subject in this category had an infection and abnormal LFTs and is also counted in that row
6 : For GI abdominal AEs occurring with a rate of at least 3% of BCX7353-treated subjects

Strong HAE Patient Demand for BCX7353:

59% of Patients Expressed High Willingness to use BCX7353 Rises to 71% with Physician Recommendation





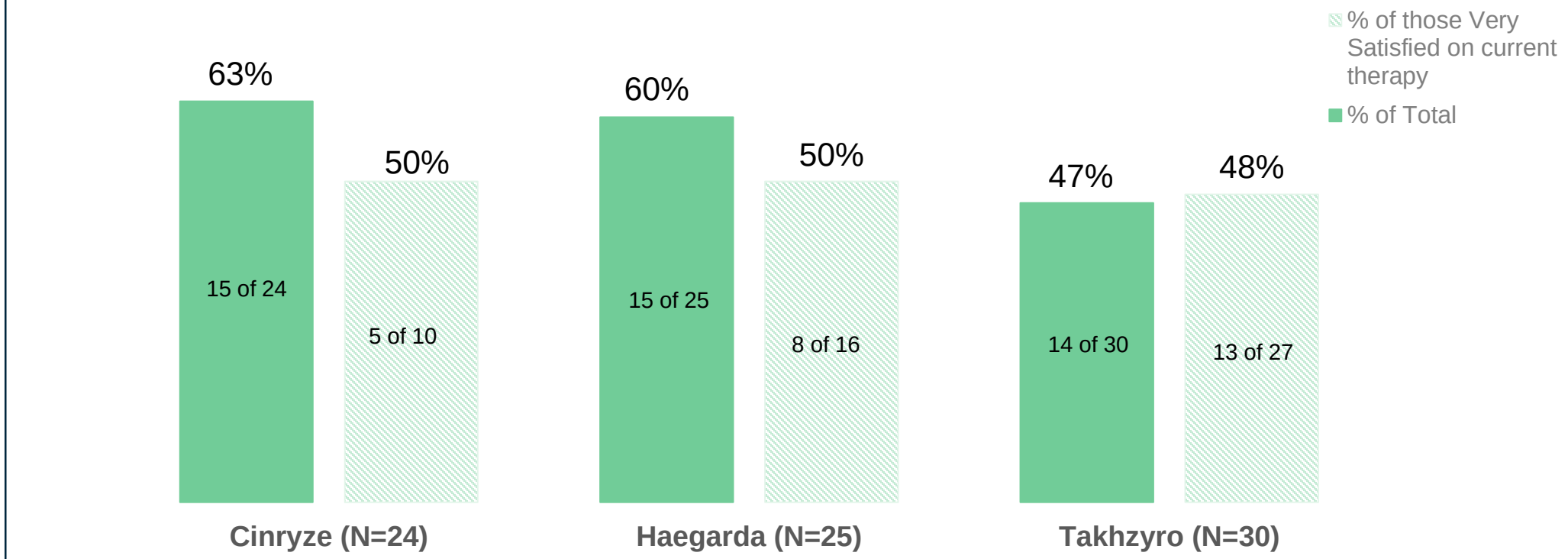
All Qualified HAE Patients (n=100)

Rated on a scale where a "0" indicates "Not at all willing", and a "10" indicates "Extremely willing"

Source: BioCryst Proprietary Market Research Study, 2019. Respondents were shown a blinded profile (Treatment X) of BCX7353, 24-week results of APeX-2

Prophylaxis Patients are Very Willing to Use BCX7353—Even Those Very Satisfied with their Current Injectable Prophylactic Treatment

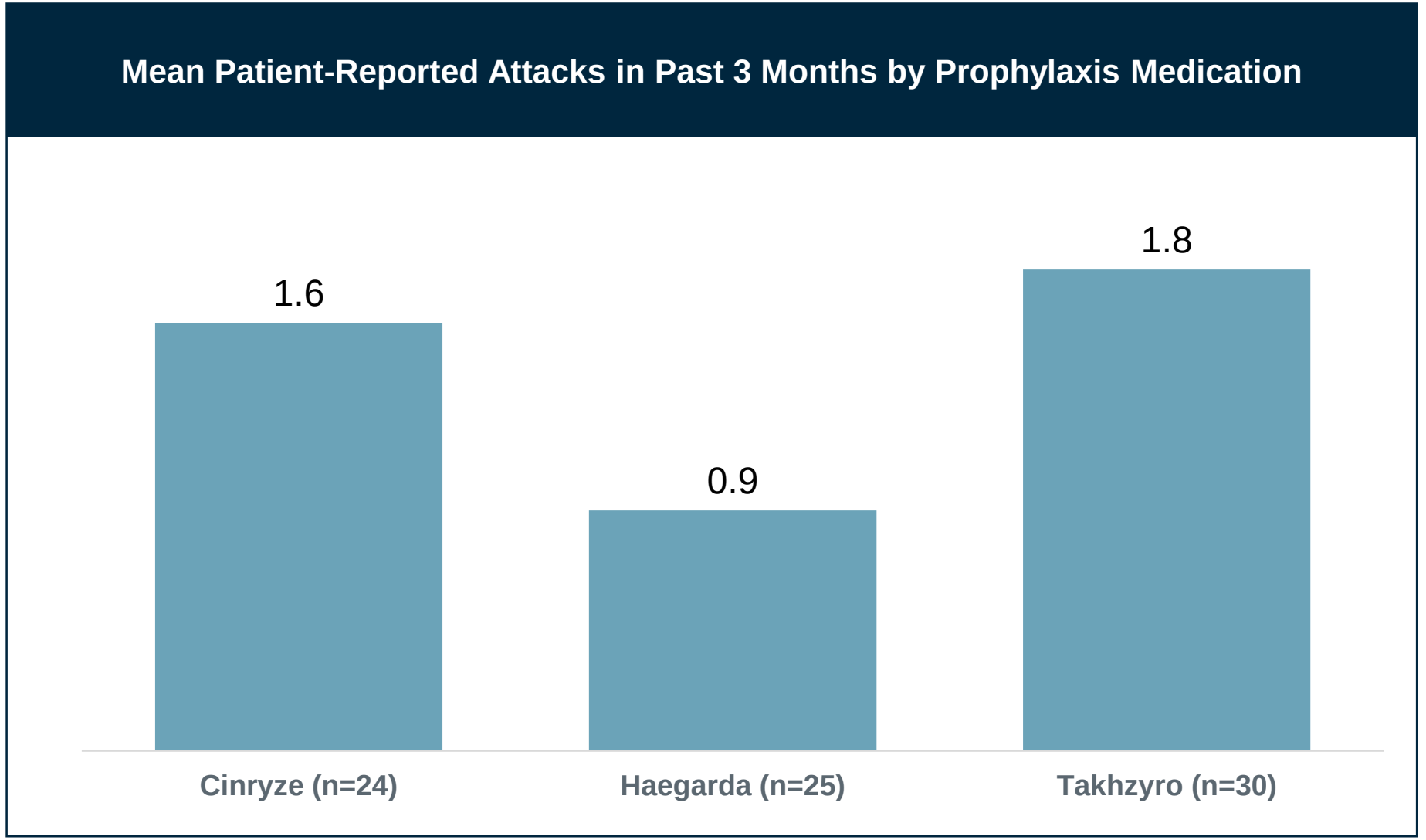
Prophylaxis Patients VERY WILLING to Use BCX7353



All Current Prophylaxis Users- "Very Willing" & "Very Satisfied" = Top 3 Box (rated 8,9,10 on 10 point scale)
Willingness rated on a scale where a "0" indicates "Not at all willing", and a "10" indicates "Extremely willing"
Satisfaction with current treatment rated on a scale where a "0" indicates "Not at all satisfied ", and a "10" indicates "Extremely satisfied"

Source: BioCryst Proprietary Market Research Study, 2019. Respondents were shown a blinded profile (Treatment X) of BCX7353, 24-week results of APeX-2

Patients Report Breakthrough Attacks with Injectable/Infused Treatments



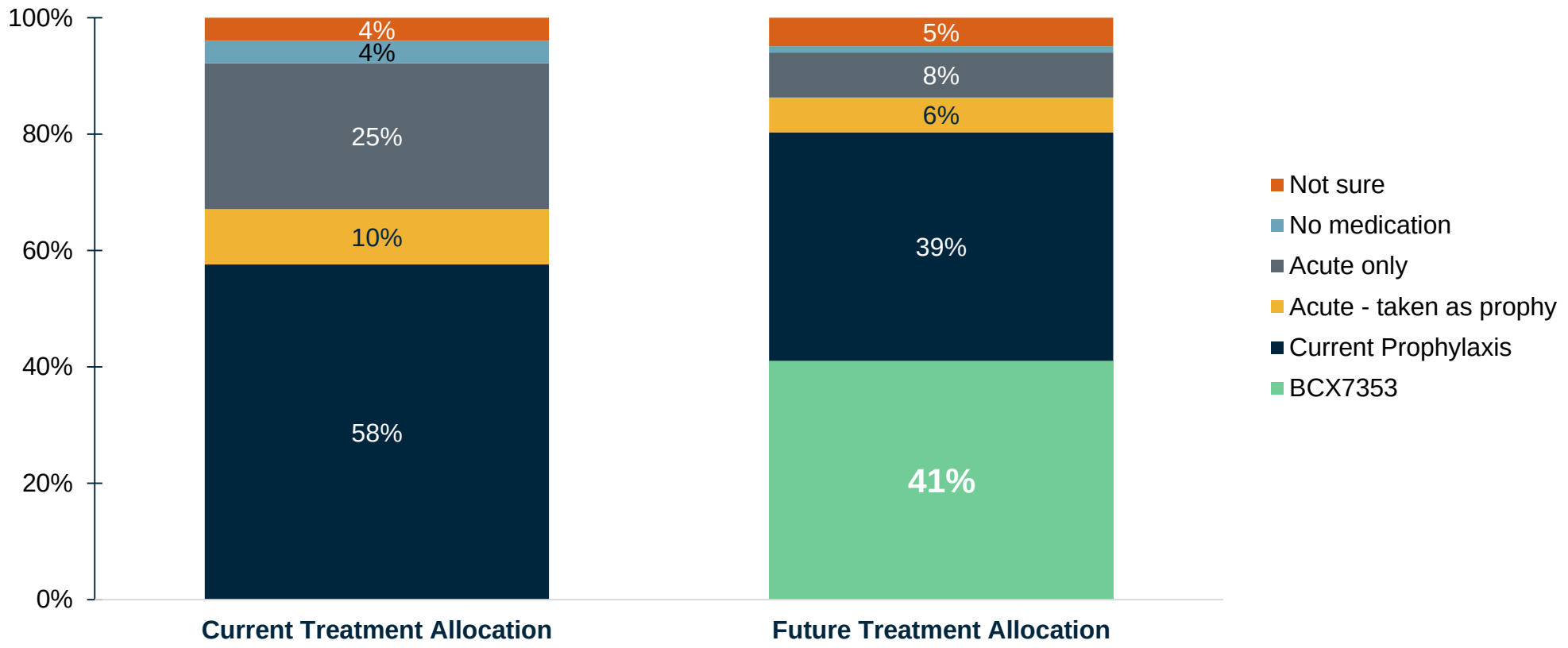
Currently Taking Medication Prophylactically

Source: BioCryst Proprietary Market Research Study, 2019.

Physicians Expect to Prescribe BCX7353 for Over 40% of HAE Patients

80% of HAE Patients Expected to be on Some Form of Prophylaxis

Patient Allocation by Medication Today and in Future with BCX7353 Available – Mean % of Patients



All Qualified Respondents (n=175)

Source: BioCryst Proprietary Market Research Study, 2019. Respondents were shown a blinded profile (Treatment X) of BCX7353, 24-week results of APeX-2, Physicians were asked to perform a patient allocation.

Factor D: Outstanding Target for Complement-mediated Diseases

Factor D is an ideal target:	Application to BCX9930 Development:
Required for the alternative pathway (AP) to work	Doses of BCX9930 that block Factor D will inhibit the AP independent of the disease setting
Target is the same in PNH, nephritis, and other AP diseases	Proof of concept in PNH provides POC for other diseases of the alternative pathway
Circulating Factor D levels are the lowest of any complement pathway enzyme	Less drug required for inhibition compared to other complement targets
Levels do not increase with inflammatory illnesses	No dose adjustment when patients get illnesses like influenza
Unique enzyme structure enables design of inhibitors with better specificity against other serine proteases	Can lead to a better safety margin

BCX9930 28-day PNH Proof of Concept Study Design

Key Outcome Measures

- **LDH, hemoglobin**
- **Safety**
- **PK**
- **PD**

	Total of 28 days of BCX9930 dosing	
	Period 1 days 1-14	Period 2 days 15-28
Subjects with PNH who are naïve to C5-INH treatments: BCX9930 monotherapy		
Cohort 1: n = up to 4	50 mg BID days 1-14	100 mg BID days 15-28
Cohort 2: n = up to 4	200 mg BID days 1-14	400 mg BID days 15-28
Subjects with poor response to C5-INH: BCX9930 plus continued C5-INH		
Cohort 1: n = up to 4	50 mg BID days 1-14	100 mg BID days 15-28
Cohort 2: n = up to 4	200 mg BID days 1-14	400 mg BID days 15-28

Subjects benefiting from study drug may continue on treatment

Cash position & 2020 guidance (in millions)

Cash & investments at December 31, 2018	$128
Cash & investments at December 31, 2019 [A]	**$138**
Senior Credit Facility [B]	$50
FY 2020 GUIDANCE	
Operating cash utilization	**$125 – 150**
Operating expenses [C]	**$135 – 160**

A - Does not include $13.9 M of cash received in February 2020 from RAPIVAB sales in Q4 2019 under our procurement contract.

B - Credit Facility was modified in February 2019 to provide an additional $20 M upon closing and the ability to draw an additional $50 M of milestone-based tranches.

C - Excludes equity-based compensation.

COVID-19 Antiviral Status Update: Galidesivir (BCX4430)

- Recurring global health crises from emerging viral infections highlight critical need for broad-spectrum antivirals in government stockpiles to protect public health

- Galidesivir has shown activity against >20 RNA viruses in 9 different families, including coronaviruses[1]
- Activity against COVID-19 coronavirus has not yet been determined

- Galidesivir was safe and generally well tolerated in two Phase 1 trials
- **Randomized, placebo-controlled trial with galidesivir in COVID-19 patients open in Brazil (NIAID-funded)**

BioCryst also working with the U.S. government to explore:

- In vitro testing against COVID-19 virus
- Increasing drug supply

$82 M of program support to date:

National Institute of Allergy and Infectious Diseases

BIOMEDICAL ADVANCED RESEARCH AND DEVELOPMENT AUTHORITY
BARDA

[1] Warren, T. K. *et al. Protection against filovirus diseases by a novel broad-spectrum nucleoside analogue BCX4430. Nature* ***508, 402-405, doi:10.1038/nature13027 (2014)***