38th Annual J.P. Morgan Healthcare Conference

Jon Stonehouse

Chief Executive Officer

January 15, 2020

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://ir.biocryst.com/financial-information/sec-filings

Delivering extraordinary
Empowering ordinary

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

The Year Ahead: 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**

Berotralstat (BCX7353):
Oral, Once Daily to Prevent HAE Attacks

APeX-2 Study – 48-week Analysis

- *Eligible HAE subjects*
- *Run-in period of 14-56 days to qualify*
- *No androgens for 28 days before screening*

→ *Randomized 1:1:1*

- *24-week pivotal period (Part 1)*
- *Placebo subjects randomized 1:1 to active at week 24*

→ *Follow-up*

→ ***48-week analysis***

Subjects Enrolled and Follow-up	**BCX7353 110 mg**	**BCX7353 150 mg**	**Placebo**	
Subjects enrolled [ITT Population]	41	40	40	
Subjects enrolled and dosed [Safety Population]	41	40	39	
Subjects who completed 24 weeks of study drug (Part 1)	37	37	34	
Randomization to active drug at conclusion of Part 1 for placebo subjects	-	-	**110 mg**	**150 mg**
Placebo subjects who were randomized (110 mg:150 mg) at 24 weeks	-	-	17	17
Subjects continuing on study, not yet reaching 48 weeks of study drug	3	1	1	1
Subjects who discontinued study drug between 24 and 48 weeks	9	6	2	3
Subjects who completed 48 weeks of study drug* [Completers Population]	25 (61%)	30 (75%)	14 (82%)	13 (76%)
Weeks of BCX7353 treatment for Completers Population	*48*	*48*	*24*	*24*
Previous Prophylactic Treatments for HAE	**BCX7353 110 mg**	**BCX7353 150 mg**	**Placebo**	
Androgens	19 (46%)	21 (53%)	25 (63%)	
C1-INH	16 (39%)	21 (53%)	16 (40%)	

** Study drug includes BXC7353 through the 48-week visit or placebo through the 24-week visit followed by BCX7353 through the 48-week visit (i.e., for 24 weeks).*

APeX-S Study – 48-week Analysis

- *Eligible HAE subjects*
- *No run-in period*
- *No androgens for 7 days before entry*

Allocated to dose

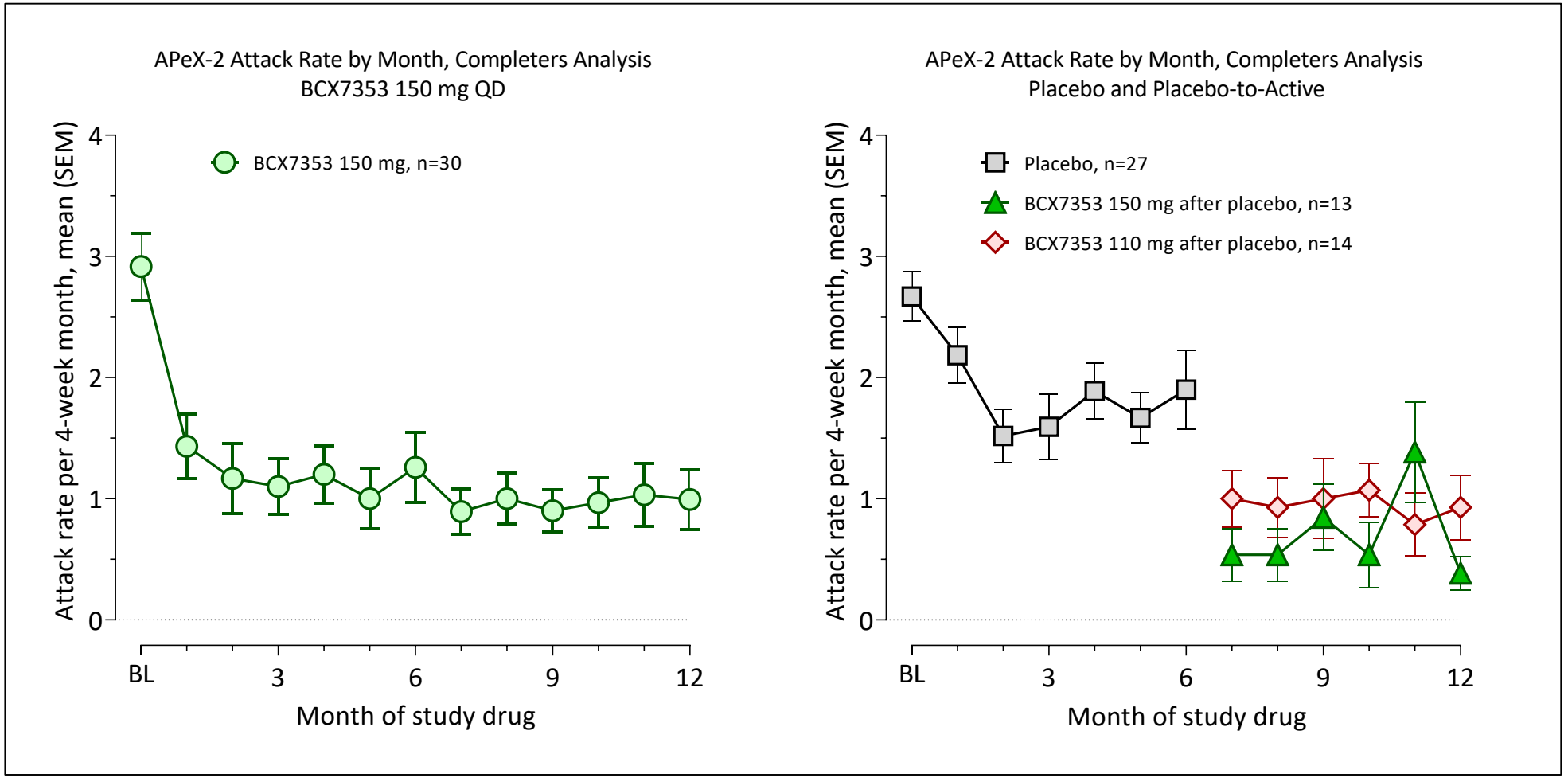
- *Daily oral BCX7353*
- *Regular clinic visits*
- *Safety assessments*
- *HAE attacks recorded*

Follow-up

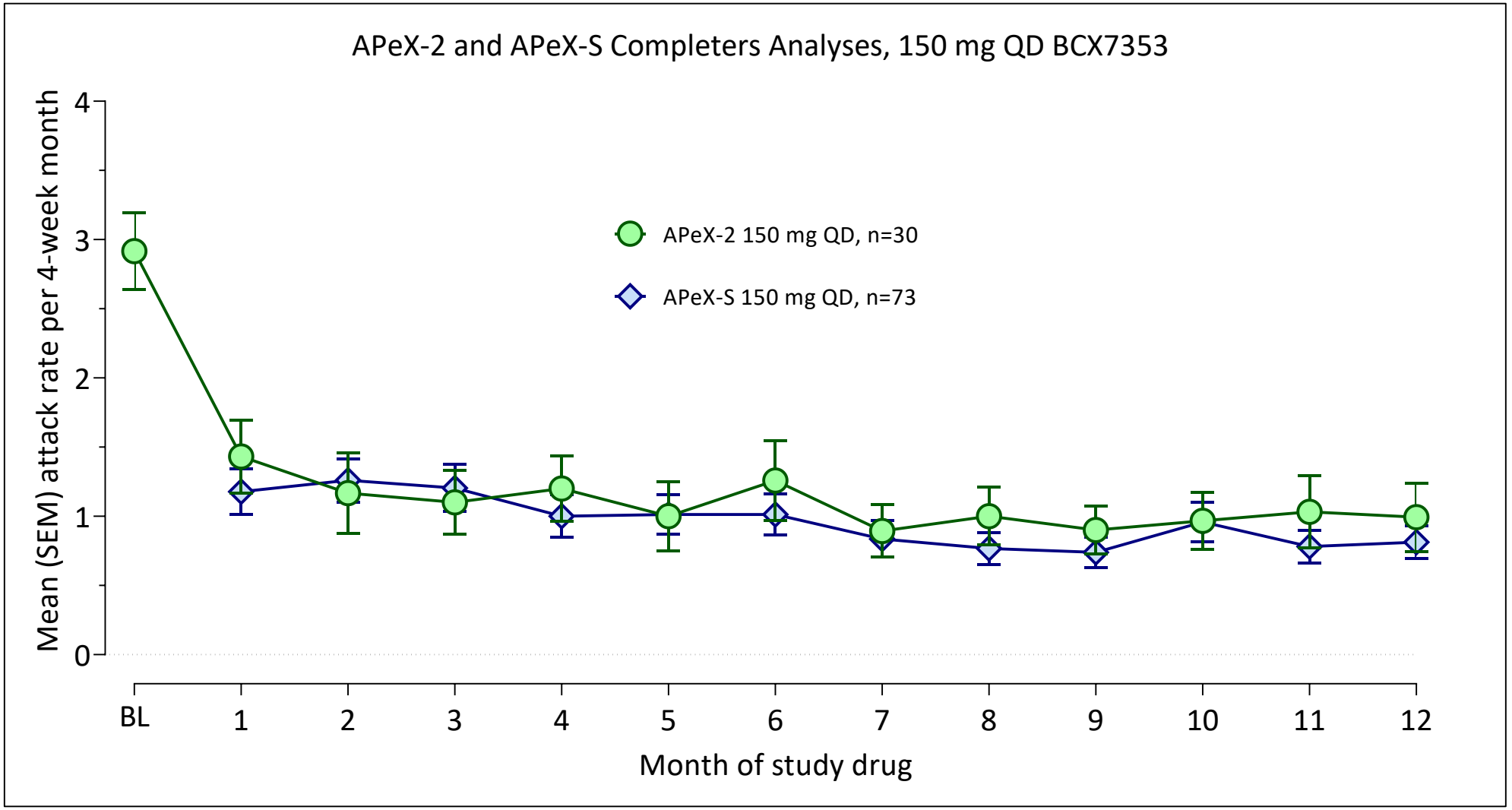
48-week analysis

Subjects Enrolled and Follow-up	**BCX7353 110 mg**	**BCX7353 150 mg**
Subjects enrolled [Safety Population]	100	127
Subjects continuing on study, not yet reaching 48 weeks of study drug	44	23
Subjects who discontinued study drug before 48 weeks	26	31
Subjects who completed 48 weeks of BCX7353 [Completers Population]	30	73
Past Prophylactic Treatment of HAE	**BCX7353 110 mg**	**BCX7353 150 mg**
Androgens	69 (69%)	84 (66%)
C1-INH	22 (22%)	32 (25%)

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

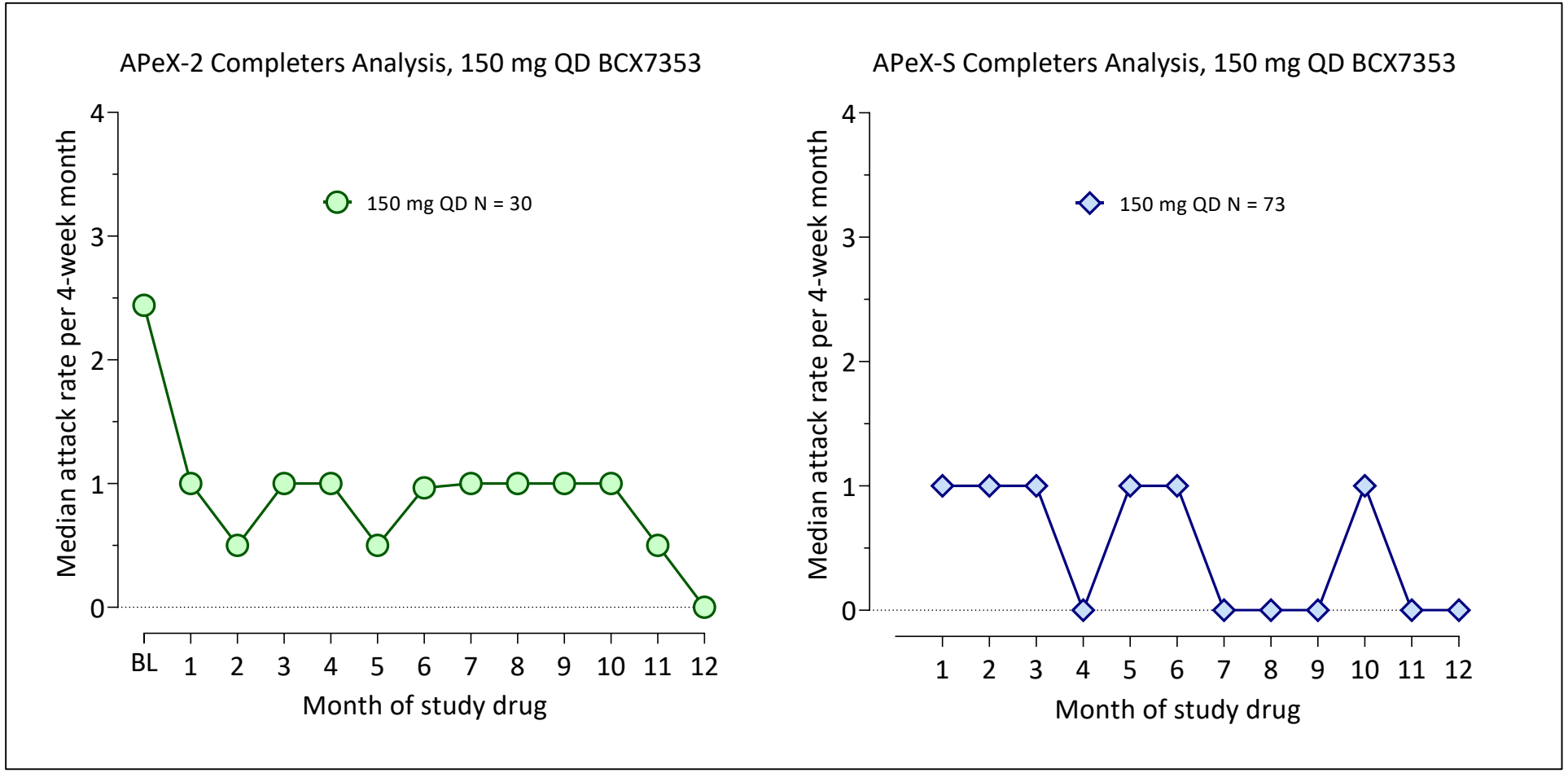


Consistent Mean Attack Rates in APeX-2 and APeX-S



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

BCX7353 Market Research Update

Robust Market Research Since APeX-2

Market Sizing

- US prevalence study using administrative claims data

US HAE Patients

- 100 quantitative, 25-minute online surveys
- 26 individual, 60- to 75-minute qualitative interviews

US Physicians

- 175 quantitative, 20-minute online surveys
- 43 individual, 60- to 75-minute qualitative interviews

US Payors

- 16 interviews with medical and pharmacy directors from insurance plans and PBMs covering >100 million lives

Source: Proprietary BioCryst studies, 2019.

Administrative Claims Analysis Estimates US HAE Population at ~10,000 Patients with ~7,500 Diagnosed & Treated

Data Source:
Administrative claims from Symphony Integrated Dataverse (IDV) from 2017-2019 for >270 million US patients

HAE Patient cohorts

1. Diagnosed and treated with HAE-specific medication
2. Diagnosed but not treated with HAE-specific medication
3. Treated with HAE-specific medication but not diagnosed

Claims Variables

- Recurring claims with HAE ICD-9/10 diagnosis codes
- Complement function and/or level tests
- Recurring claims for HAE-specific medications

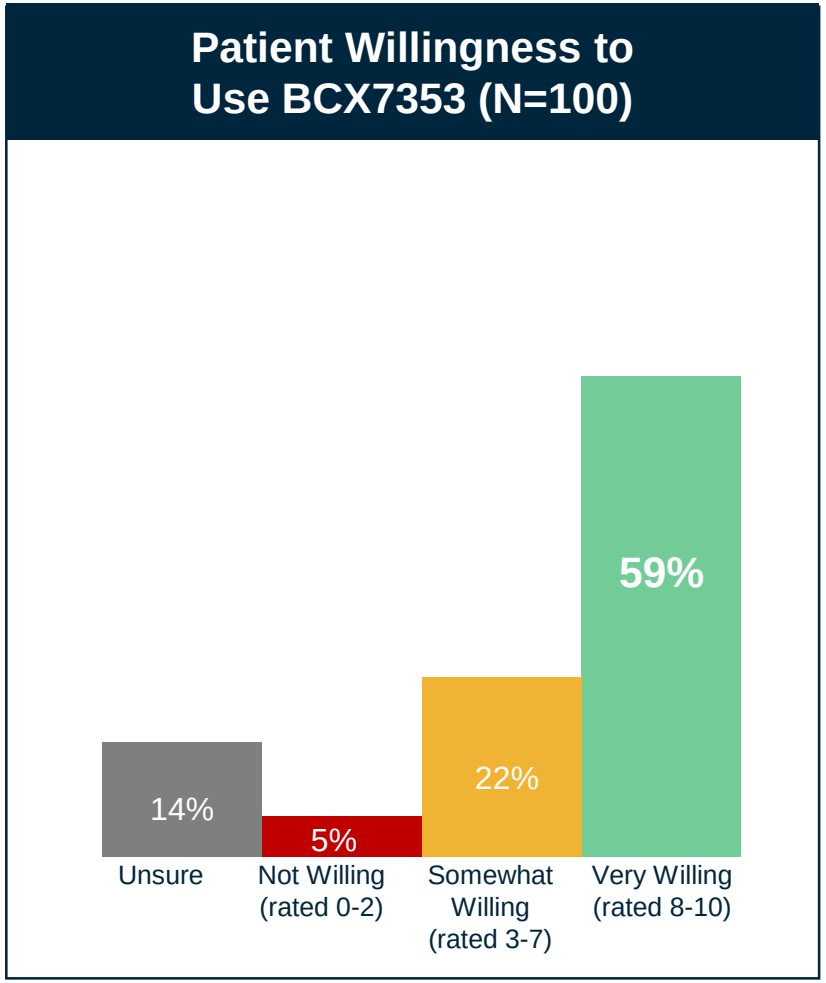
National projections*

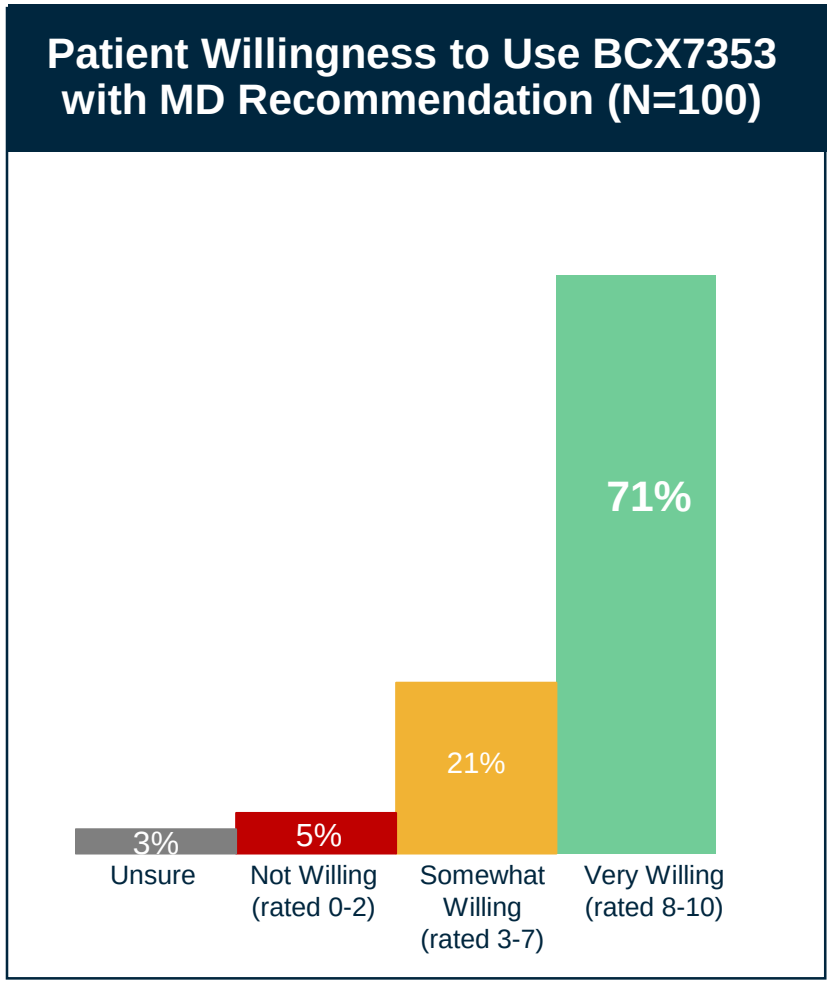
1. ~7,500 patients diagnosed and treated
2. ~1,700 patients diagnosed but not treated
3. ~600 patients treated but not diagnosed

Source: Proprietary BioCryst study, 2019. *Projections based on total US population and demographics

Strong HAE Patient Demand for BCX7353:

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





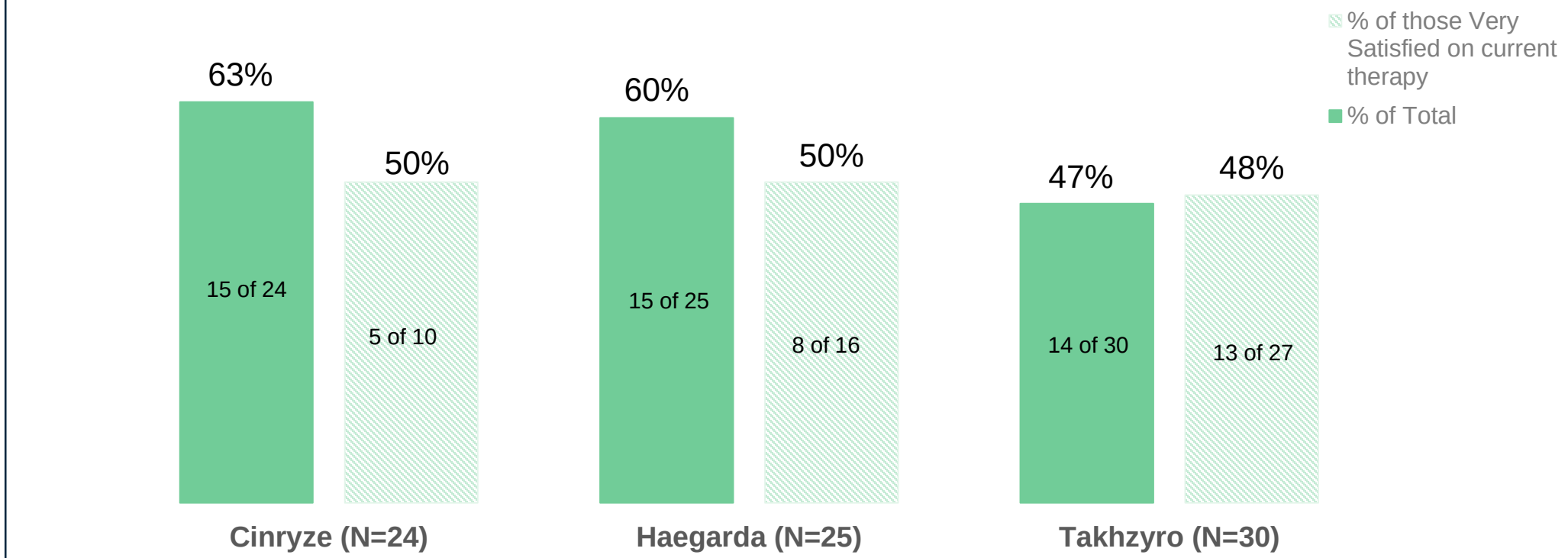
All Qualified HAE Patients (n=100)

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

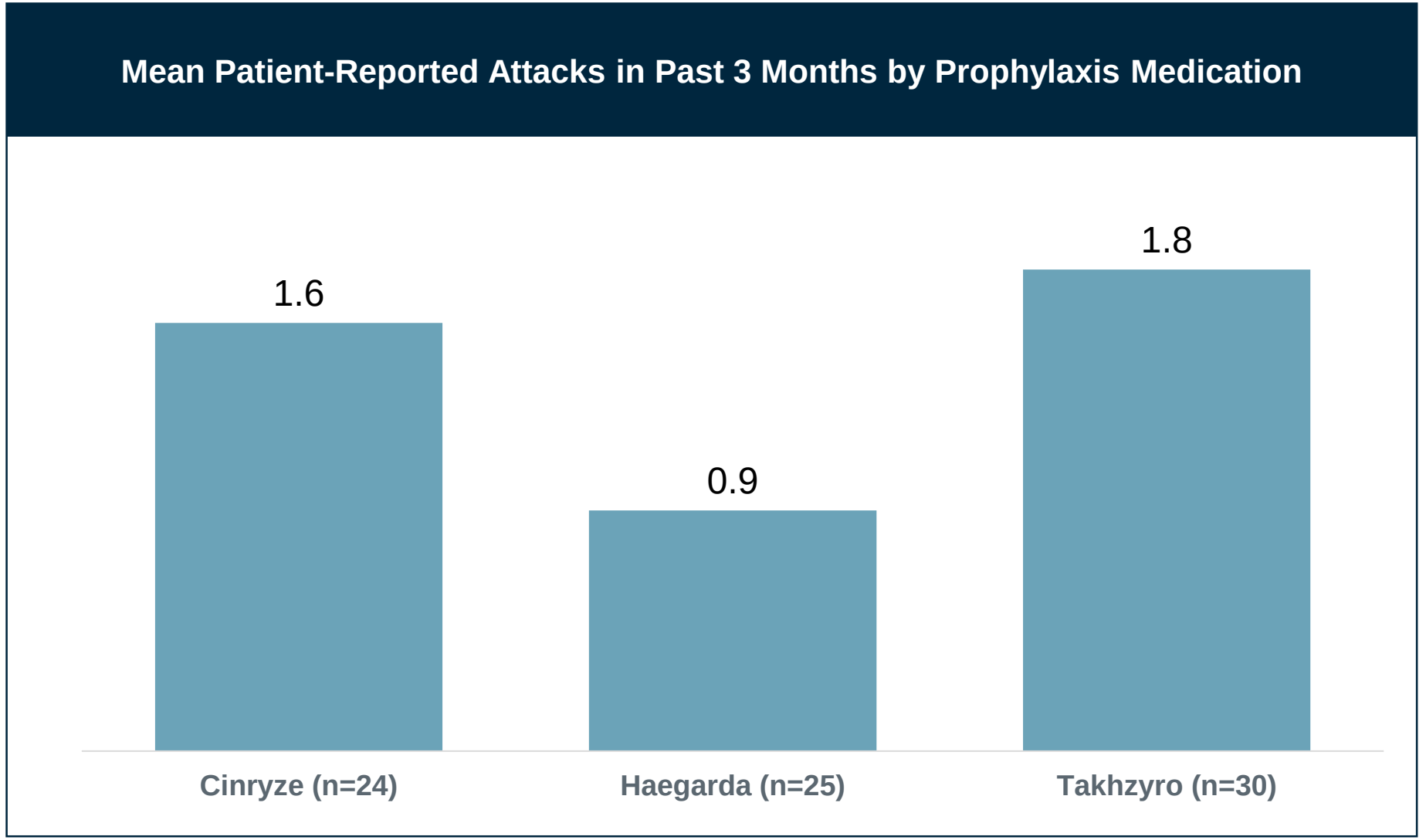
Prophylaxis Patients VERY WILLING to Use BCX7353



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



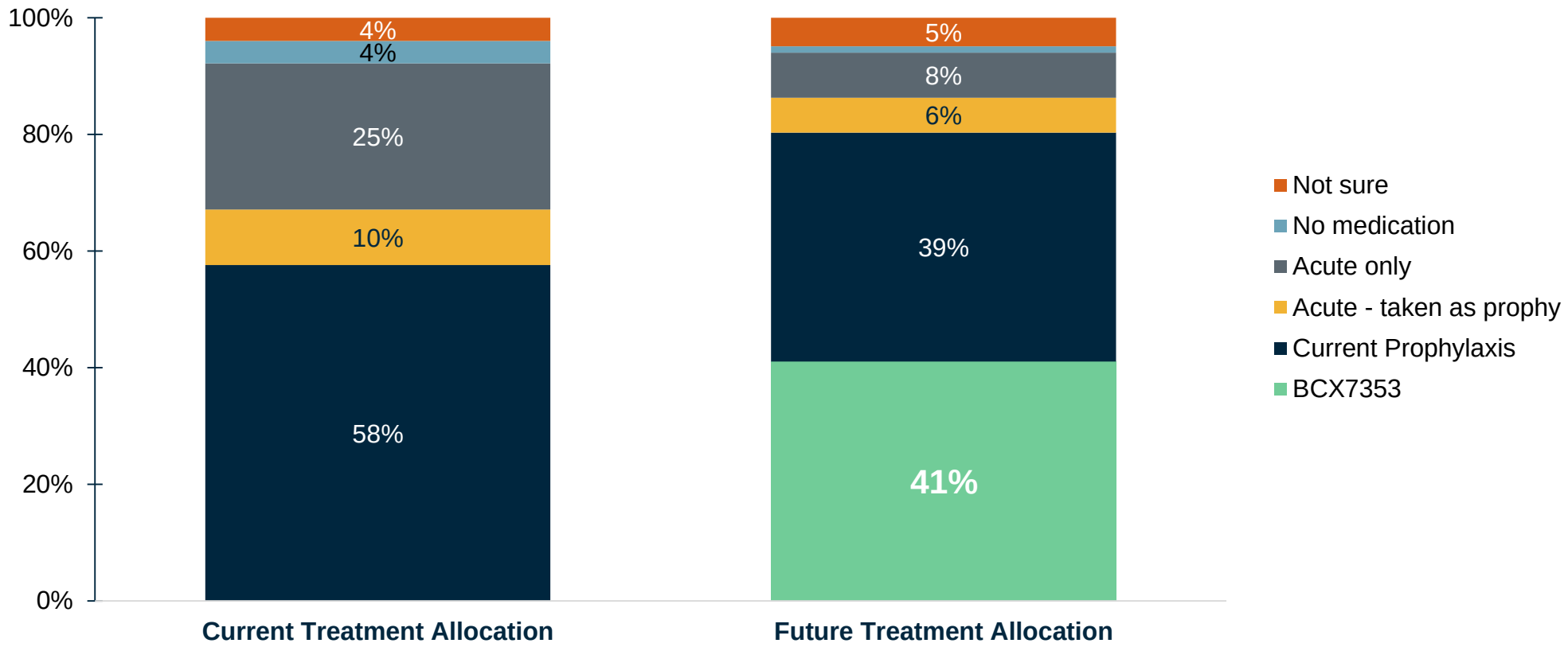
Currently Taking Medication Prophylactically

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

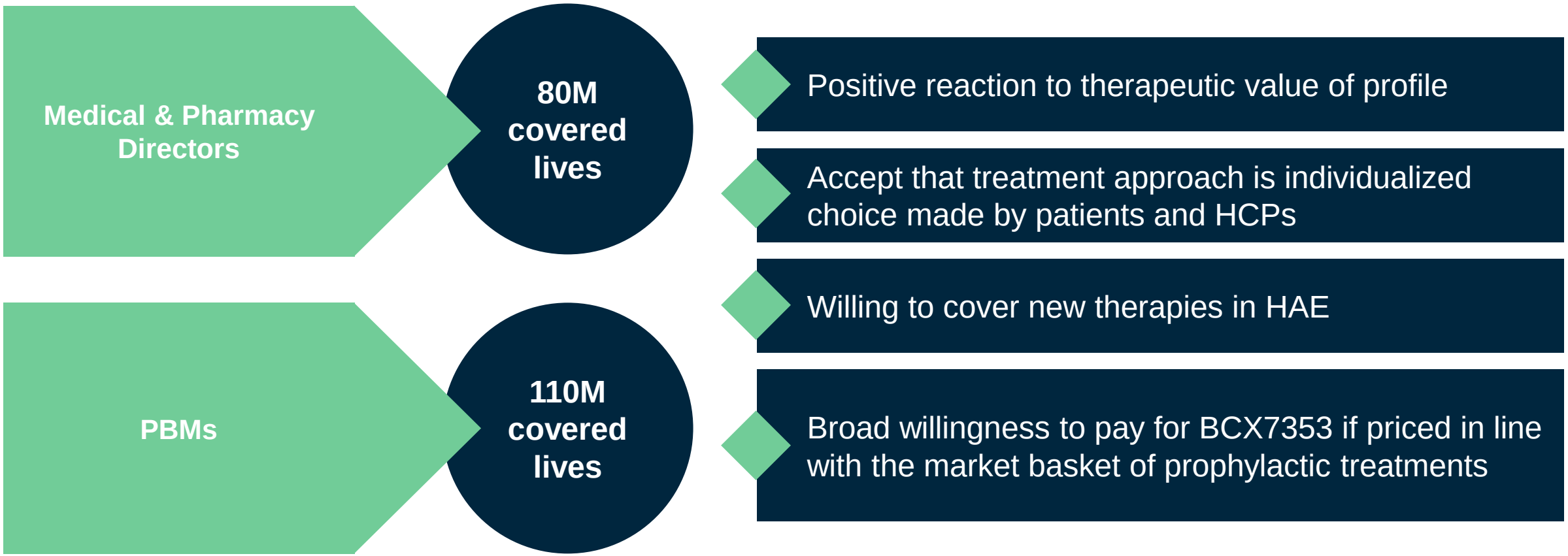
Patient Allocation by Medication Today and in Future with BCX7353 Available – Mean % of Patients



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.

US Payors Anticipate Providing Coverage for Berotralstat



Source: BioCryst Proprietary Research, 2019. Sample included 5 national insurance plans, 7 regional plans, 2 IDNs, and 2 national PBMs. Respondents were shown a blinded profile (Treatment X) of BCX7353, 24-week results of APeX-2.

Berotralstat for HAE Prophylaxis: Data Supports Global Peak Market Opportunity >$500M

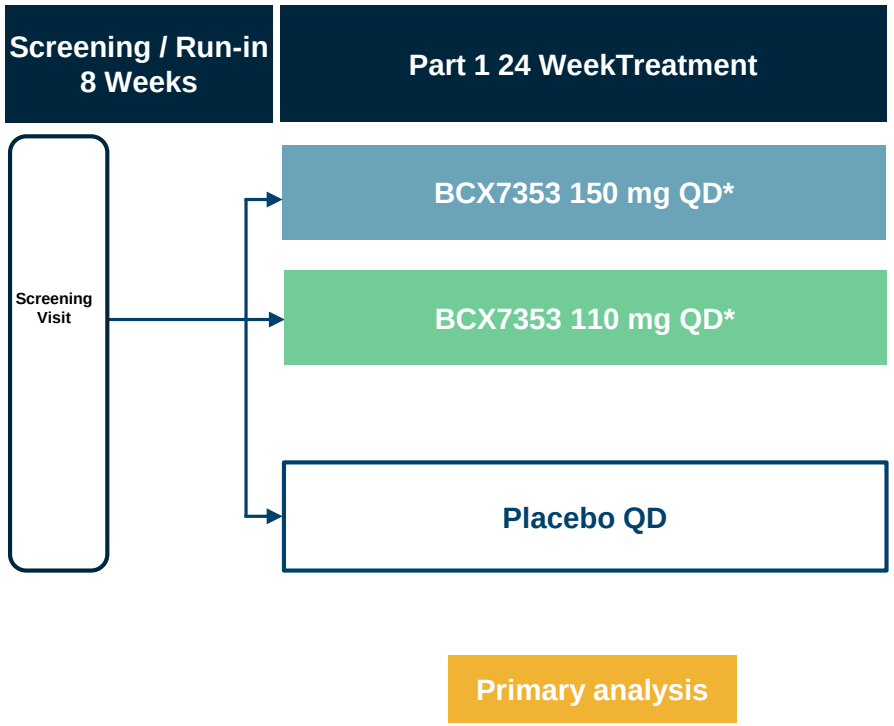
Clinical Data	Prevalence	Treatment Paradigm
Consistent, clinically meaningful benefit demonstrated through 48 weeks Safe and generally well-tolerated	~10,000 (US) HAE Patients ~7,500 diagnosed and treated	Physicians expect shift to ~80% prophylaxis

Strong Demand for BCX7353 Product Profile and Benefit
Overall, 60-70% of patients very willing to use
Physicians intending to prescribe to >40% of patients
Payors acknowledge therapeutic value and broad willingness to pay

Multiple Potential Global Approvals in 2020-2021

APeX-J – Primary Efficacy Endpoint was Met for Berotralstat 150 mg

Total Enrollment: 19 (7 at 150 mg, 6 at 110mg, 6 placebo)

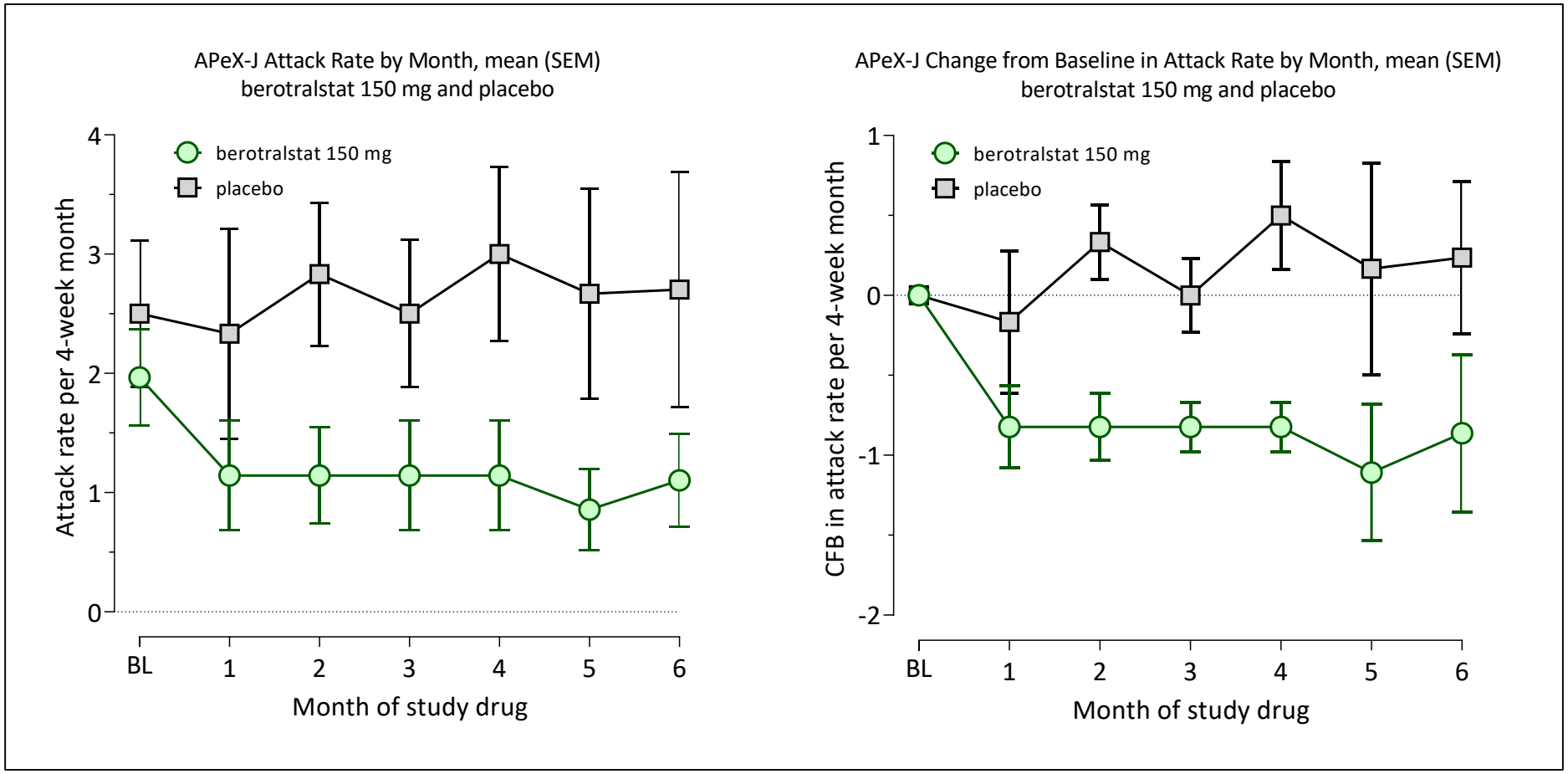


Primary endpoint: expert-confirmed angioedema attacks, rate/month*

Arm	N	Rate	Attack rate ratio active/placebo (95% CI)	Percent reduction from placebo (95% CI)	P value
Berotralstat 150 mg	7	1.11	0.51 (0.33, 0.80)	49.1 (20.4, 67.5)	0.003
Berotralstat 110 mg	6	1.64	0.75 (0.50, 1.14)	24.6 (-14.0, 50.1)	0.181
Placebo	6	2.18	-	-	-

** Statistical analysis is based on a negative binomial regression model. The number of expert-confirmed events is included as the dependent variable, the treatment is included as a fixed effect, baseline expert-confirmed event rate is included as a covariate, and the logarithm of duration on treatment is included as an offset variable.*

Berotralstat 150 mg Shows Consistent, Sustained Reduction in Attacks Over 24 Weeks

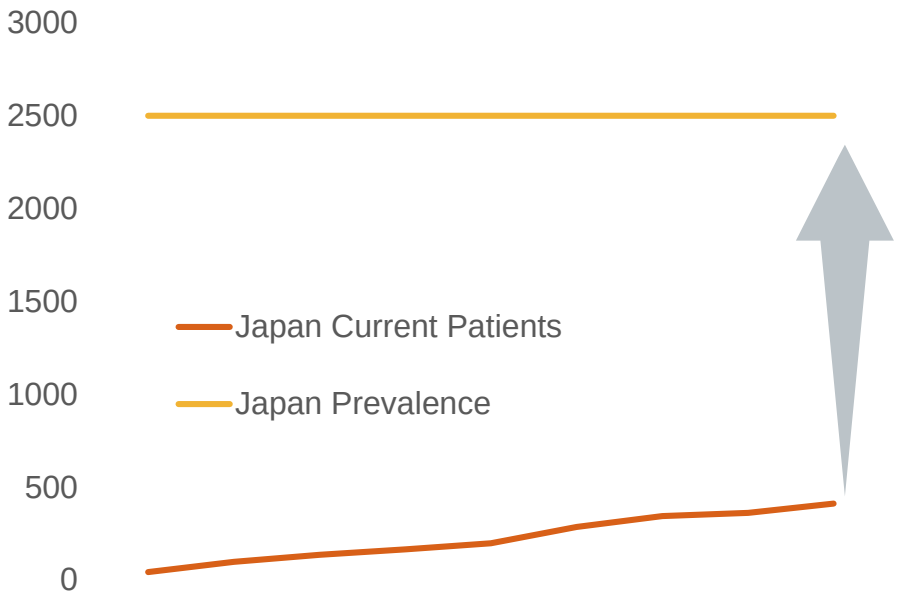


Overall Safety Summary:
Berotralstat was Safe and Generally Well Tolerated

Treatment-emergent (TE) Adverse Events (AEs) or Discontinuations (DCs) due to TEAEs	Berotralstat 110 mg	Berotralstat 150 mg	Placebo
	N = 6	N = 7	N = 6
Any Drug-Related TEAEs	2 (33.3%)	2 (28.6%)	2 (33.3%)
Drug-Related Serious TEAEs	0	0	0
Drug-Related Grade 3 or 4 TEAEs	0	0	0
Any Drug-Related Abdominal GI TEAE	2 (33.3%)	1 (14.3%)	1 (16.7%)
Most Common[1] Drug-Related TEAEs			
Abdominal discomfort	1 (16.7%)	0	1 (16.7%)
DCs due to TEAEs	0	0	1 (16.7%)[2]

[1] Occurring in >1 subject
[2] One placebo subject discontinued due to urticaria

Unique Market Opportunity in Japan



Source: HAE-J, Presentation: HAE Global Conference, 2016

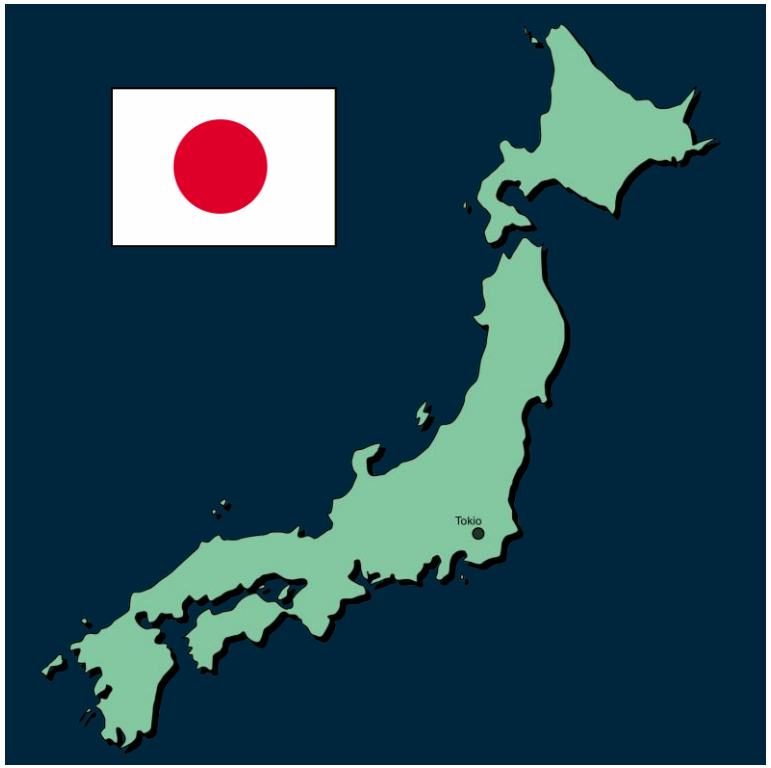
Japanese Market Growth Potential

- Berotralstat would be 1st approved prophylactic HAE therapy in Japan
- Active KOL base of treating physicians with strong interest in new therapies for patients
- Lower awareness of disease and lack of standard-of-care treatments have limited diagnosis rates compared to US
- Very active patient advocacy groups increasing awareness in HAE prophylaxis

Berotralstat for HAE Prophylaxis: Japanese Partnership with Torii

Non-dilutive Capital + Access to Unique Market with Large Unmet Need

- $42 million in upfront and milestones
  - $22 million upfront
  - Up to $20 million with approval + threshold pricing
  - Royalties from mid-teens up to potentially 40%
- Proven, committed partner
- Sakigake designation could enable Japan to be 1st global approval
- JNDA on-track for Q1 2020



BCX9930
Oral Factor D Inhibitor for Complement-mediated Diseases

Over $10 Billion Global Market Opportunity

Significant pipeline potential for a differentiated oral complement inhibitor

*The only marketed complement inhibitors are **IV-infused****

2

*2018 **sales from 3 indications,** 55% ex-US***

$3.6 billion

Orphan indications,** each with >$1B potential sales*

7+

*** SOLIRIS® (eculizumab) is indicated for in paroxysmal nocturnal hemoglobinuria (PNH), atypical hemolytic uremic syndrome (aHUS), generalized myasthenia gravis (gMG), and neuromyelitis optica (NMO) spectrum disorder; and ULTOMIRIS® (ravulizumab-cwvz) is indicated for PNH and aHUS; both products are manufactured by Alexion Pharmaceuticals, Inc.**

**** SOLIRIS® 2018 sales for PNH, aHUS, and gMG, reported 2/4/19**

***** Additional current and potential orphan indications for complement inhibitors include, but are not limited to, 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 Phase 1 Trial Design & Progress

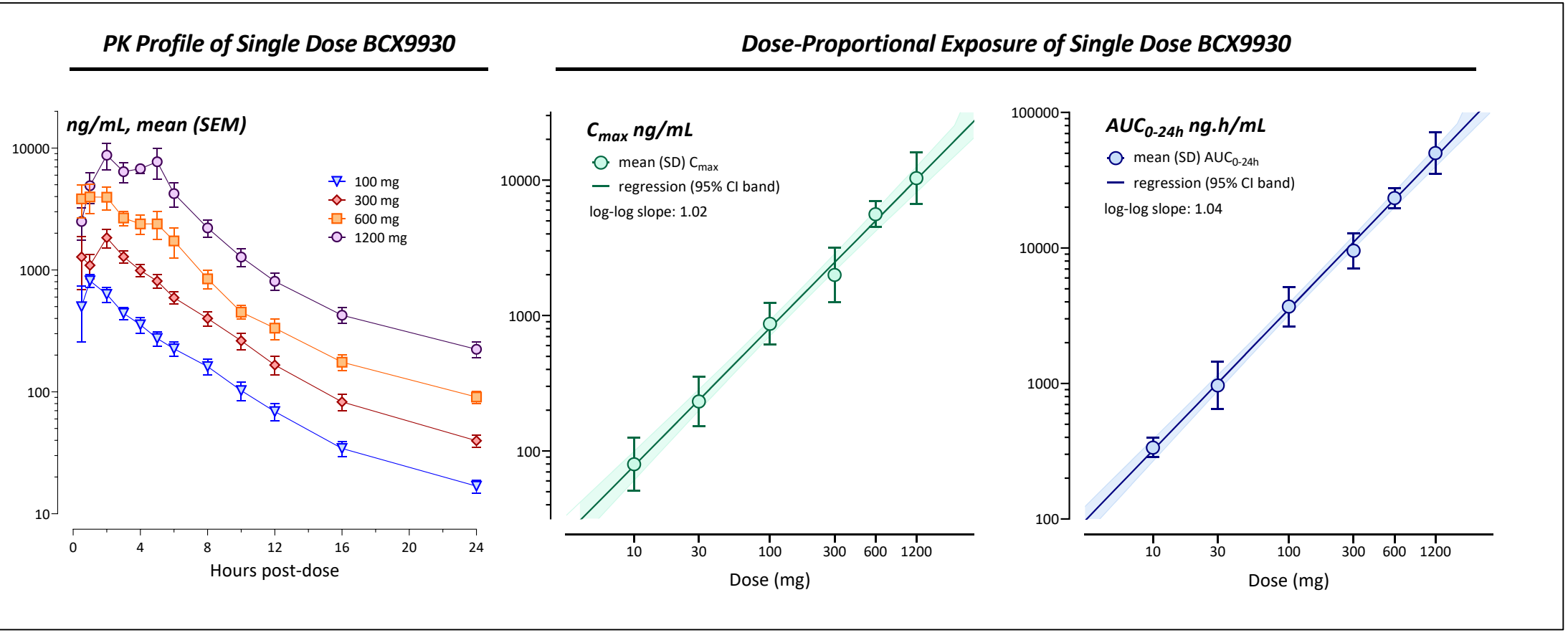
Part 1 – Single ascending dose
- Healthy subjects
- PK & PD
- Safety and tolerability
- 8 subjects per cohort
  6:2 active : placebo
- 6 dose levels
- Completed

Part 2 – Multiple ascending dose
- Healthy subjects
- PK & PD
- Safety and tolerability
- 12 subjects per cohort
  10:2 active : placebo
- Multiple dose levels
- Ongoing

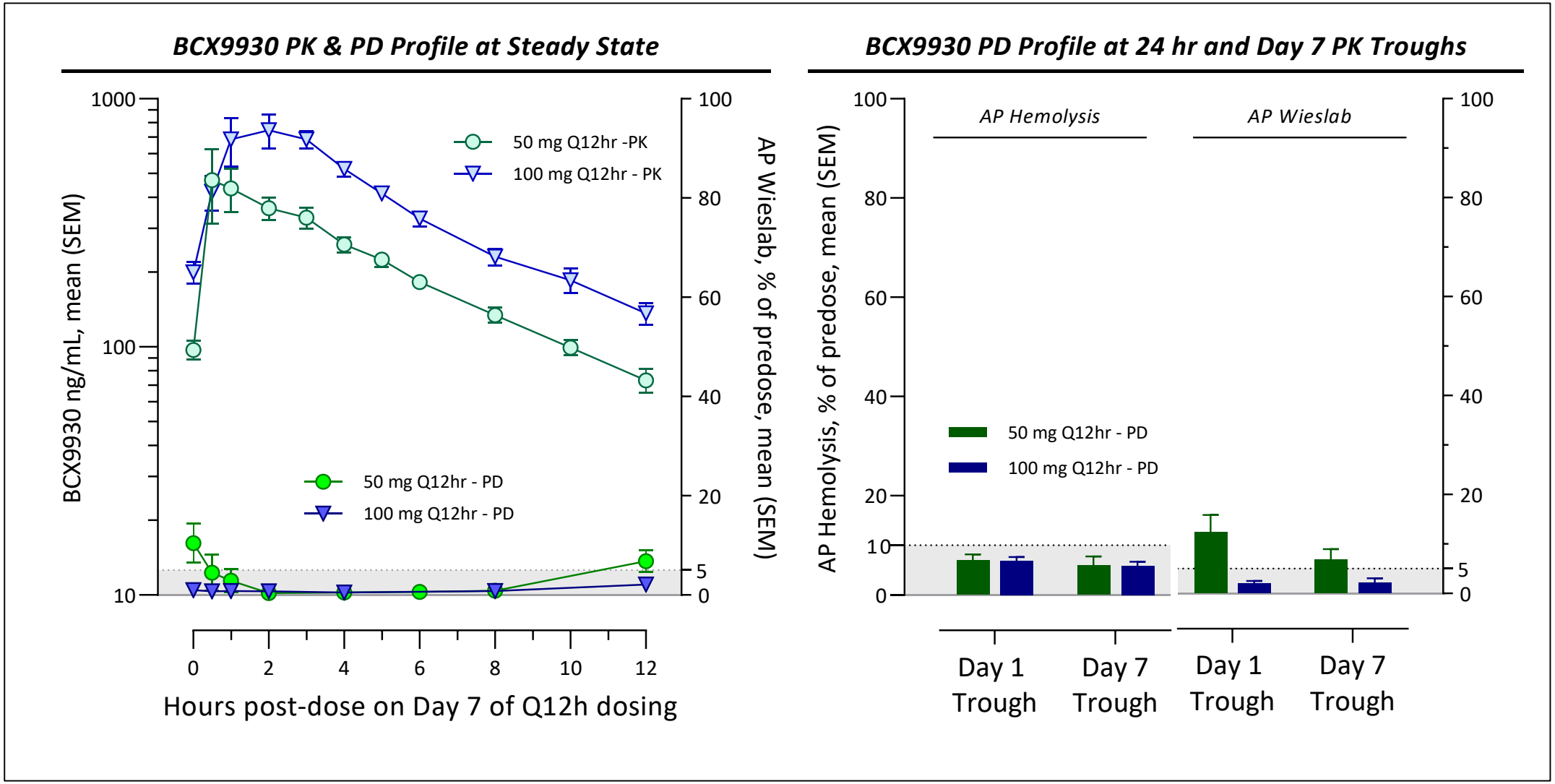
Part 3 – Proof of concept in PNH patients
- Poor responders to eculizumab or ravulizumab, or naïve to treatment
- Up to 16 patients total
- Multiple dose levels

- Part 1 : SAD completed with cohorts from 10 to 1200 mg
- Part 2 : Three MAD cohorts completed
  50 mg Q12hr x 7 days and 100 mg Q12hr x 7 days with concomitant antibiotic
  50 mg Q12hr x 14 days with vaccination
- Part 3 : PNH proof of concept data expected 1H 2020

Single Dose PK Profile of Oral BCX9930 in Healthy Subjects



Steady State PK and PD with Q12hr Dosing of BCX9930



BCX9930 Phase 1 Trial: Summary

PK/PD

- Linear, dose-proportional exposure
- Dose-related suppression of alternative pathway of complement functional activity
- > 95% inhibition of alternative pathway in AP Wieslab assay at 100 mg Q12hr through 7 days of dosing

Safety & Tolerability

- Safe and generally well-tolerated at all doses
- No serious adverse events
- No safety signals in routine monitoring of:
  - Vital signs, ECGs, or laboratory evaluations of hematology, coagulation, urinalysis, or clinical chemistry that included hepatic and renal
- Benign rash in majority of MAD subjects that was self-limited and resolved within a median of 5 days of onset

BCX9930: Program Update

- 12 healthy subjects (10 active, 2 placebo) vaccinated against *Neisseria meningitidis*, and then dosed with BCX9930 50 mg Q12h for a planned 14 days, in order to answer key scientific questions
- In the event of rash, subjects were to be discontinued from dosing if the rash was more than limited in extent
- Subjects with limited rash could continue on study drug per protocol
- Subjects who developed a rash could consent to skin biopsy

Scientific question	Result	Conclusion
Did antibiotic contribute to incidence of rash?	Benign rash observed in 7 subjects	NO— Antibiotic not likely a contributing factor
Was the clinical pattern of rash different in absence of antibiotic?	Same pattern was observed – clinically benign	NO— Antibiotic not likely a contributing factor
Did the rash change/worsen in subjects who continued dosing with study drug?	Same pattern clinically, median of 5 days duration	NO— In 2 healthy volunteers who continued dosing, rash resolved on-drug
Did biopsy results contribute to the understanding of the rash?	Majority of subjects with rash consented to biopsy - superficial perivascular dermatitis was found with no evidence of vasculitis	YES— Confirmed as benign

Proof of Concept Data in PNH Patients Expected 1H 2020

BCX9250
Oral ALK-2 Inhibitor for Fibrodysplasia Ossificans Progressiva (FOP)

Fibrodysplasia Ossificans Progressiva (FOP)

Devastating disease; no treatments available

- Rare disease that affects approximately 1 in 2 million people worldwide
- Irregular formation of bone in muscles, tendons or soft tissue
- Currently no approved treatments for FOP
- Phase 1 trial underway in healthy volunteers to assess safety, data expected 2H 2020

Cash Position & 2019 Guidance (in Millions)

- Added $100 M to balance sheet in Q4 2019 -

Cash & investments at December 31, 2018	$128
Proforma cash & investments at September 30, 2019 [A]	**$170**
Senior Credit Facility	$50
FY 2019 GUIDANCE	
Operating cash utilization	**$105 – 130**
Operating expenses [B]	**$120 – 145**

A – Proforma cash balance adjusts Sept. 30, 2019 cash balance for $100 of net proceeds from fourth quarter 2019 equity raises and upfront payment from Torii for Japanese commercial rights to berotralstat.

B - Excludes equity-based compensation.

The Year Ahead: 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**