Fourth Quarter 2019 Results Call
Corporate Update & Financial Results

March 5, 2020

Forward-Looking Statements

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Agenda

- Corporate Update:

  Jon Stonehouse – President, Chief Executive Officer

- Global Commercial Update:

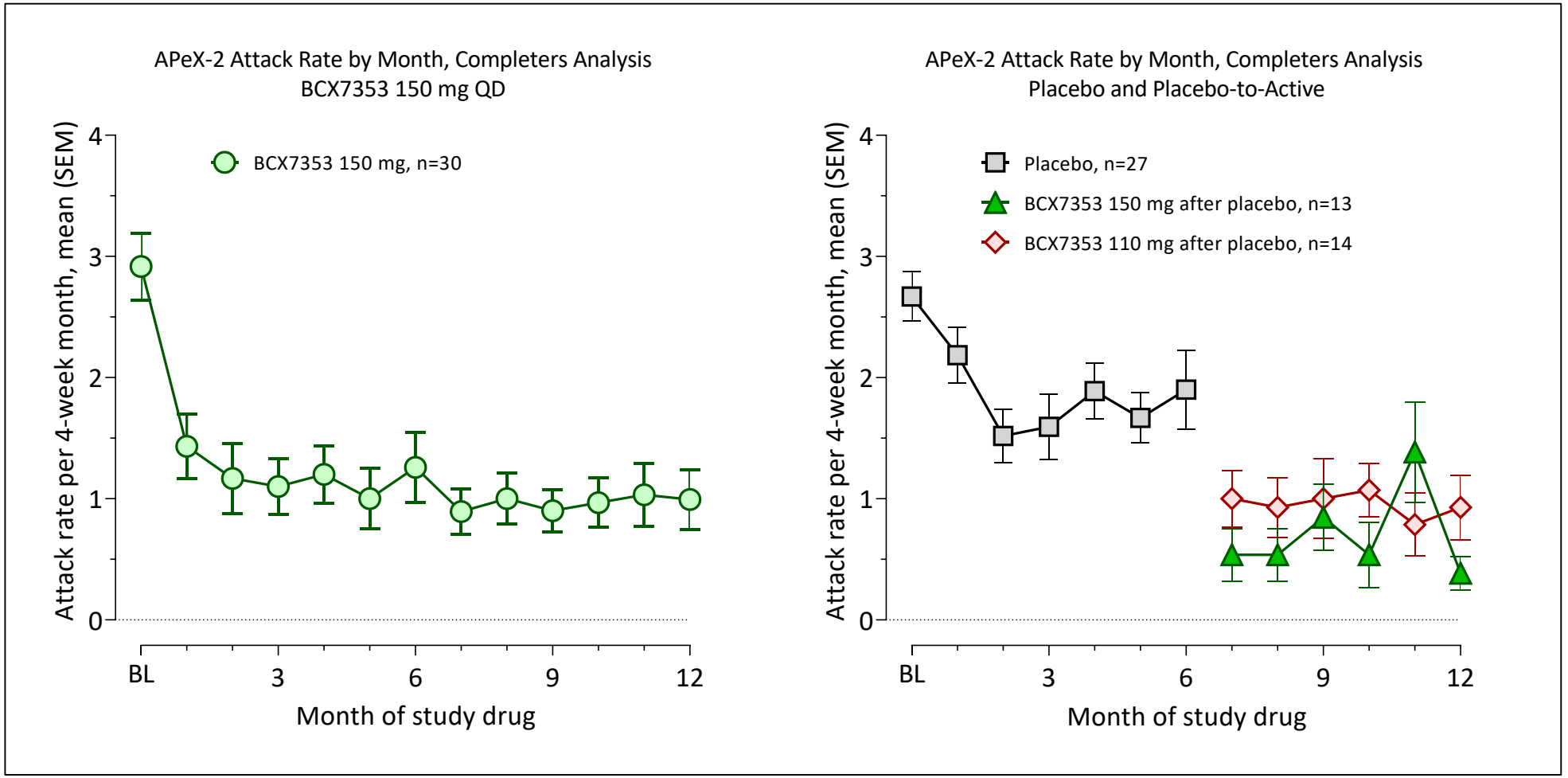
  Charlie Gayer– Chief Commercial Officer

- Clinical Update

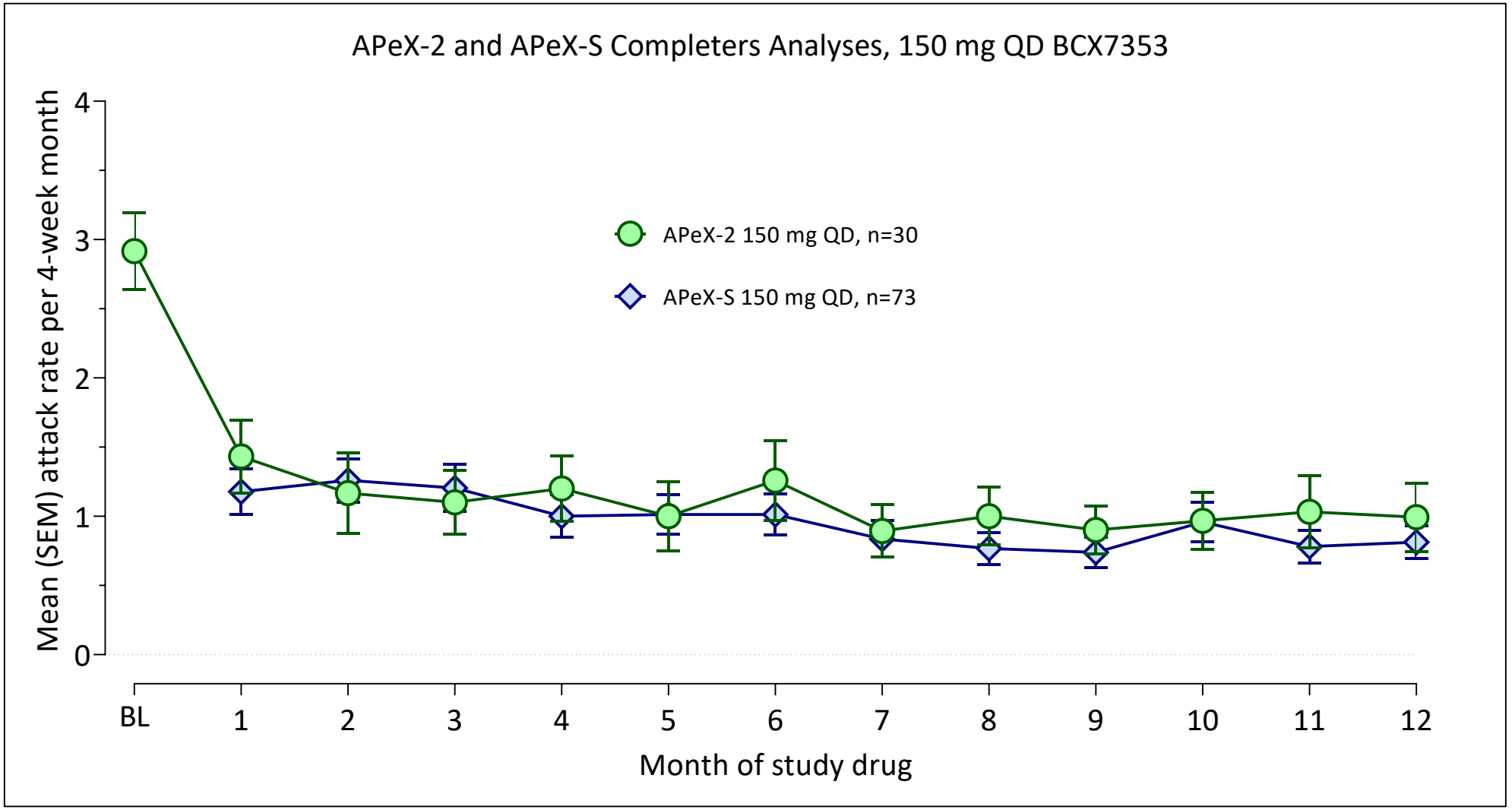
  Dr. Bill Sheridan – Chief Medical Officer

- Summary and Q&A

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

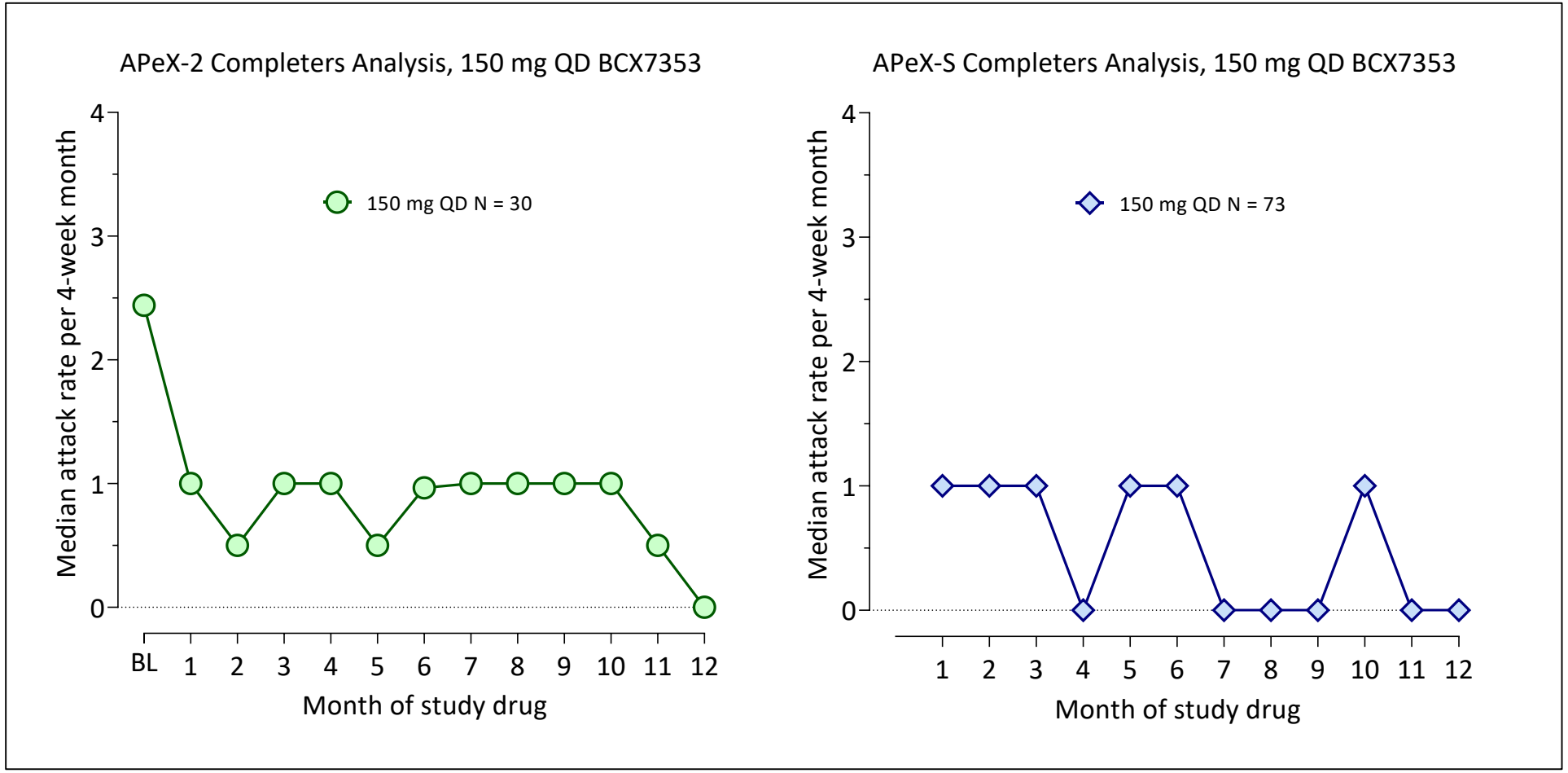


Consistent Mean Attack Rates in APeX-2 and APeX-S



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

Global Commercial Update:

Charlie Gayer– Chief Commercial Officer

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

Large, Quantitative Market Research Studies with US Patients and HAE-treating Physicians in July 2019 with 24-week APeX-2 Profile

100 HAE Patients

- 25-minute online survey
- Age 18+, diagnosed with Type I or II HAE
- Currently treating HAE or not currently treating and has 1+ attack every 3 months
- 50% recruited from HAEA patient organization
- 50% recruited via social media and online panels

175 HAE-Treating Physicians

- 20-minute online survey
- Allergist/Immunologist (n=100)
- Other specialty (n=75)
- Actively treats 2+ Type I or II HAE patients per year
- Study average = 7.6 patients/year
- Recruited via email and online panels

Physicians **in this study treat 1,300 HAE patients representing over 10% of US HAE patients**

Source: Proprietary BioCryst study, 2019.

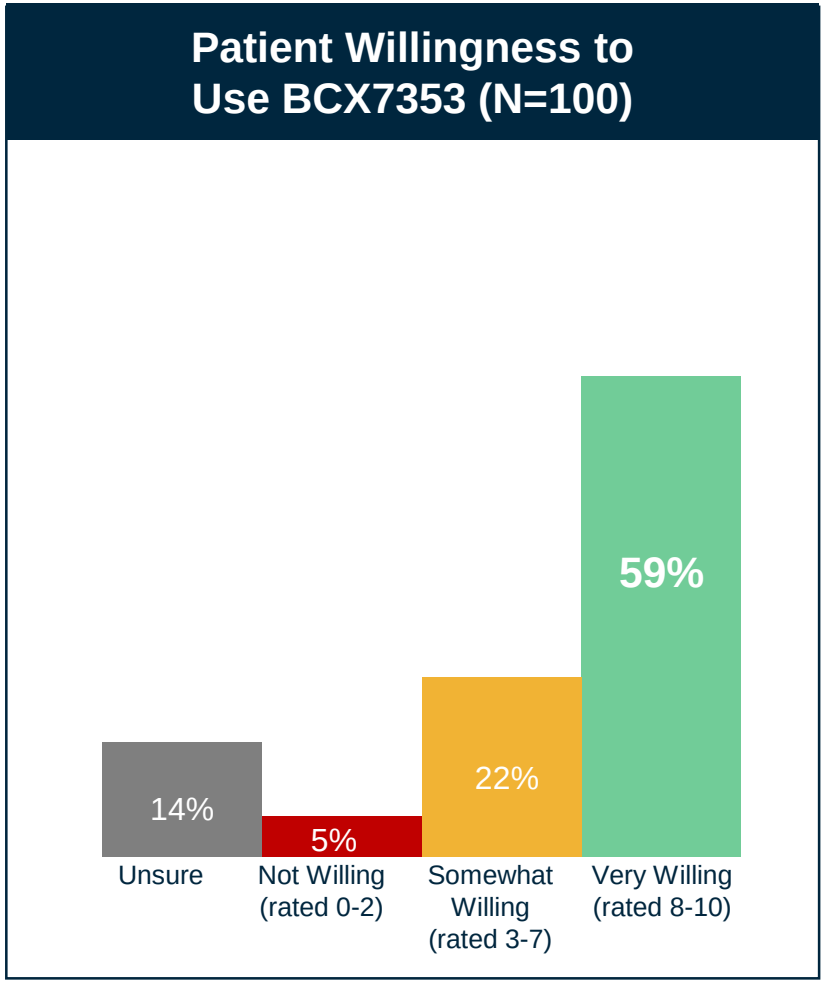
Respondents Viewed a Blinded Profile of BCX7353 Based on 24-week Results from APeX-2

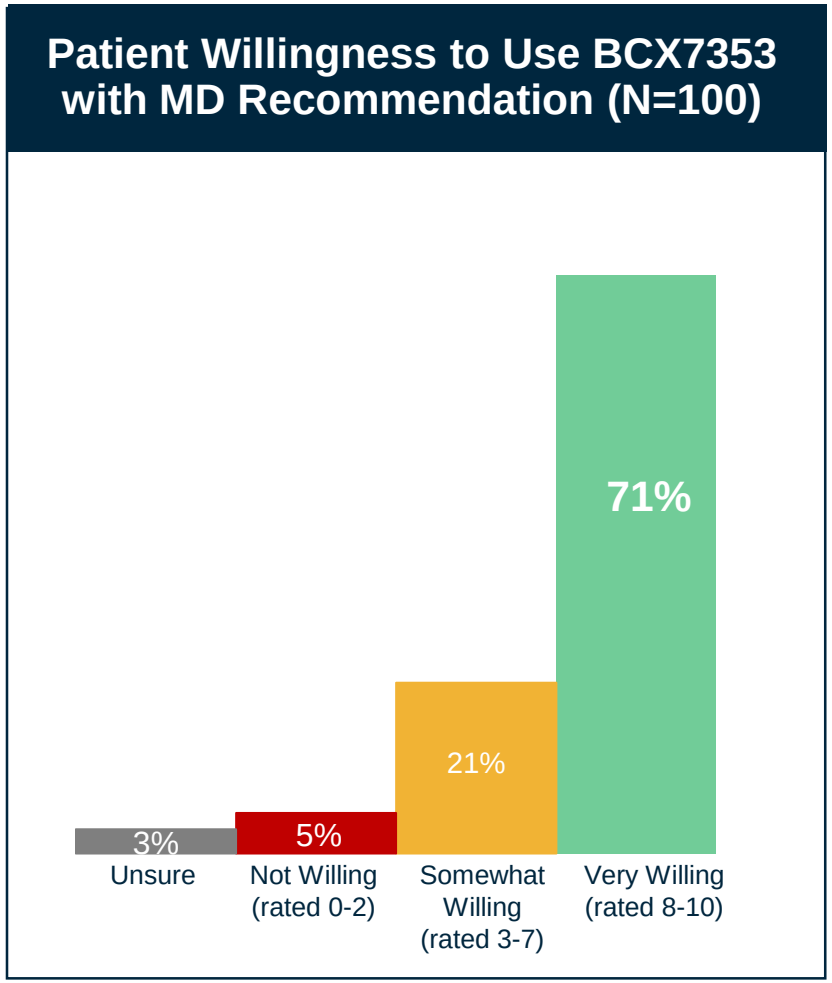
Indication	Prophylactic treatment of HAE for patients 12 years and above
Dosage	Take 1 capsule by mouth once per day
Clinical trial design	Patients who were experiencing an average of 3 HAE attacks per month took Treatment X or a placebo (an inactive drug often used in clinical trials) for 6 months
Efficacy	Patients taking Treatment X had 44% fewer HAE attacks overall than patients taking a placebo during the 6-month clinical trial Half (50%) of patients taking Treatment X reduced their number of HAE attacks by 70% or more between the beginning and end of the trial About 1 in 4 patients (23%) taking Treatment X reduced their number of HAE attacks by 90% or more between beginning and end of the trial
Safety and tolerability	Adverse events from Treatment X were generally mild and similar to placebo The most common side effects experienced more often with Treatment X were short episodes of mild diarrhea or vomiting experienced by about 10% of patients

Source: Proprietary BioCryst study, 2019.

Strong HAE Patient Demand for BCX7353:

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





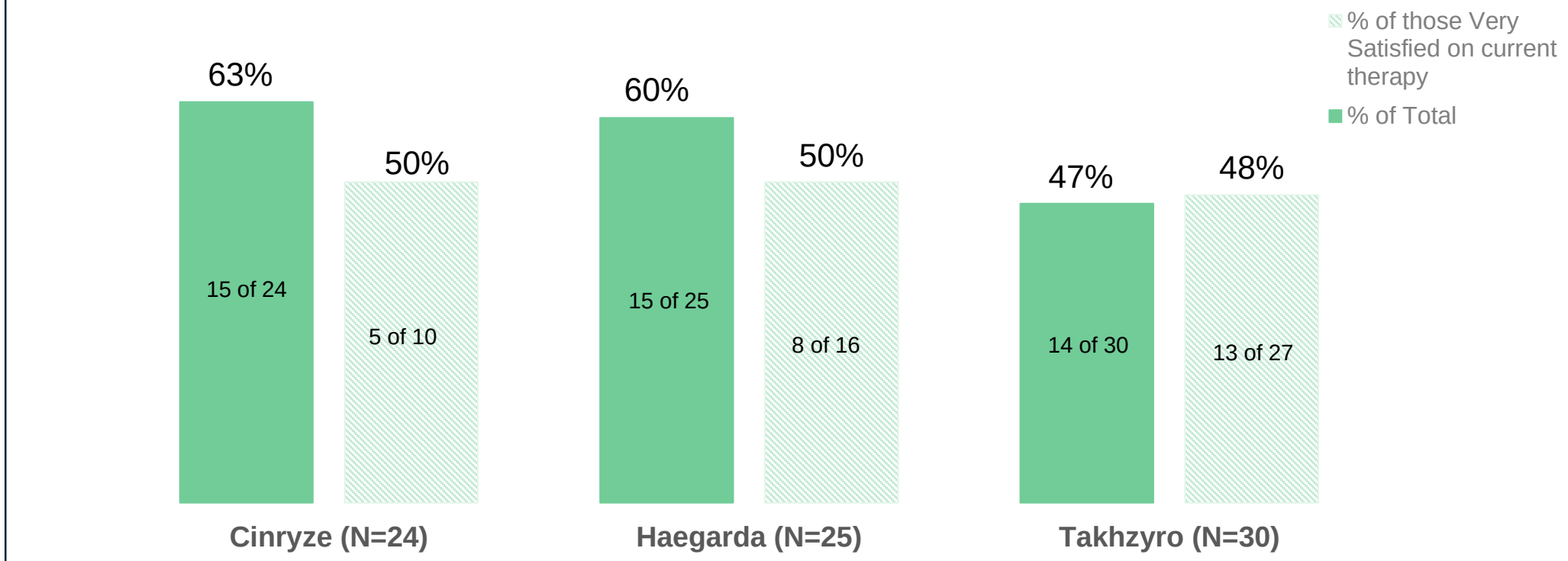
All Qualified HAE Patients (n=100)

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

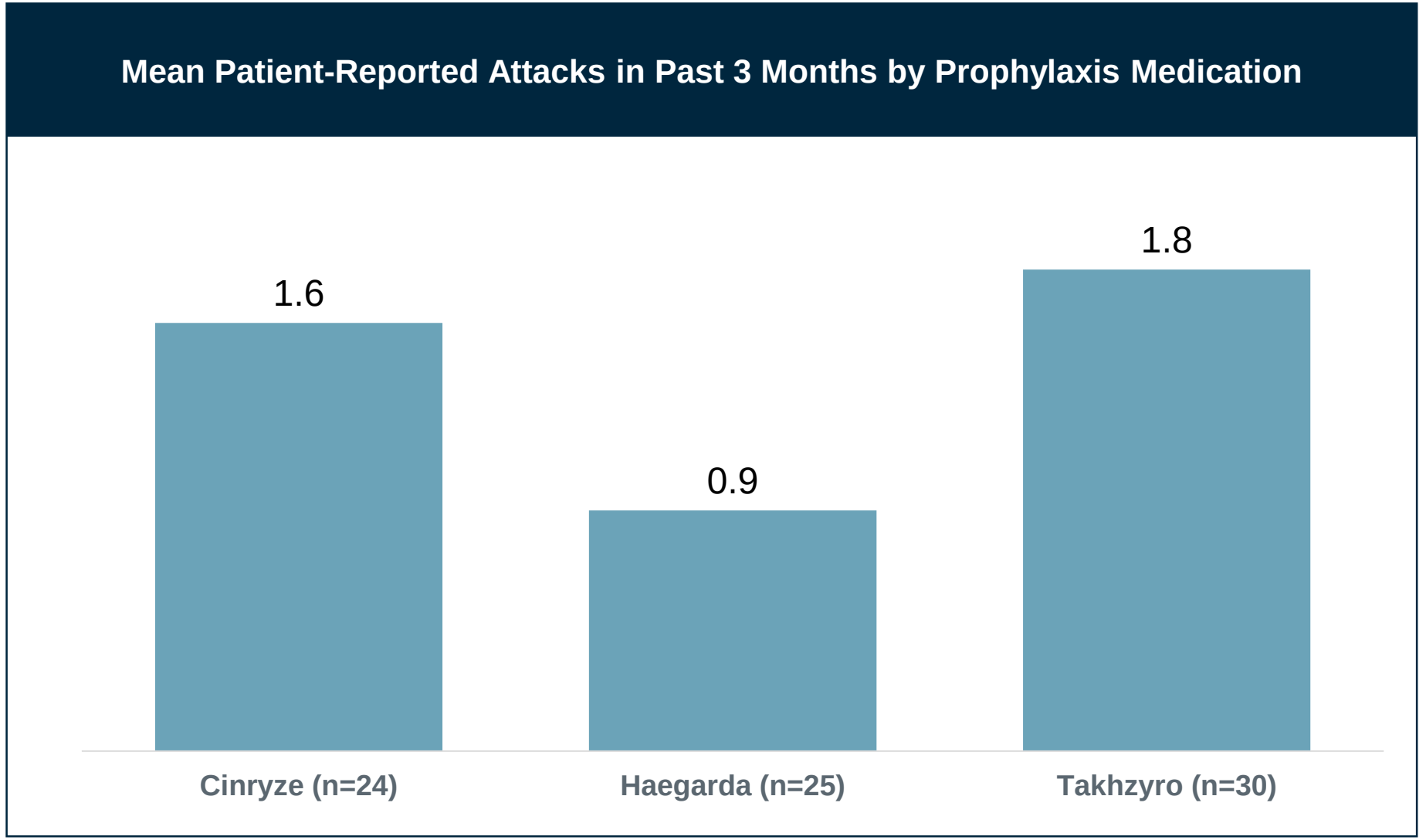
Prophylaxis Patients VERY WILLING to Use BCX7353



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.

Patients Are Coping With Their Injectable Therapies

They Want the Ease that Only Berotralstat can Offer

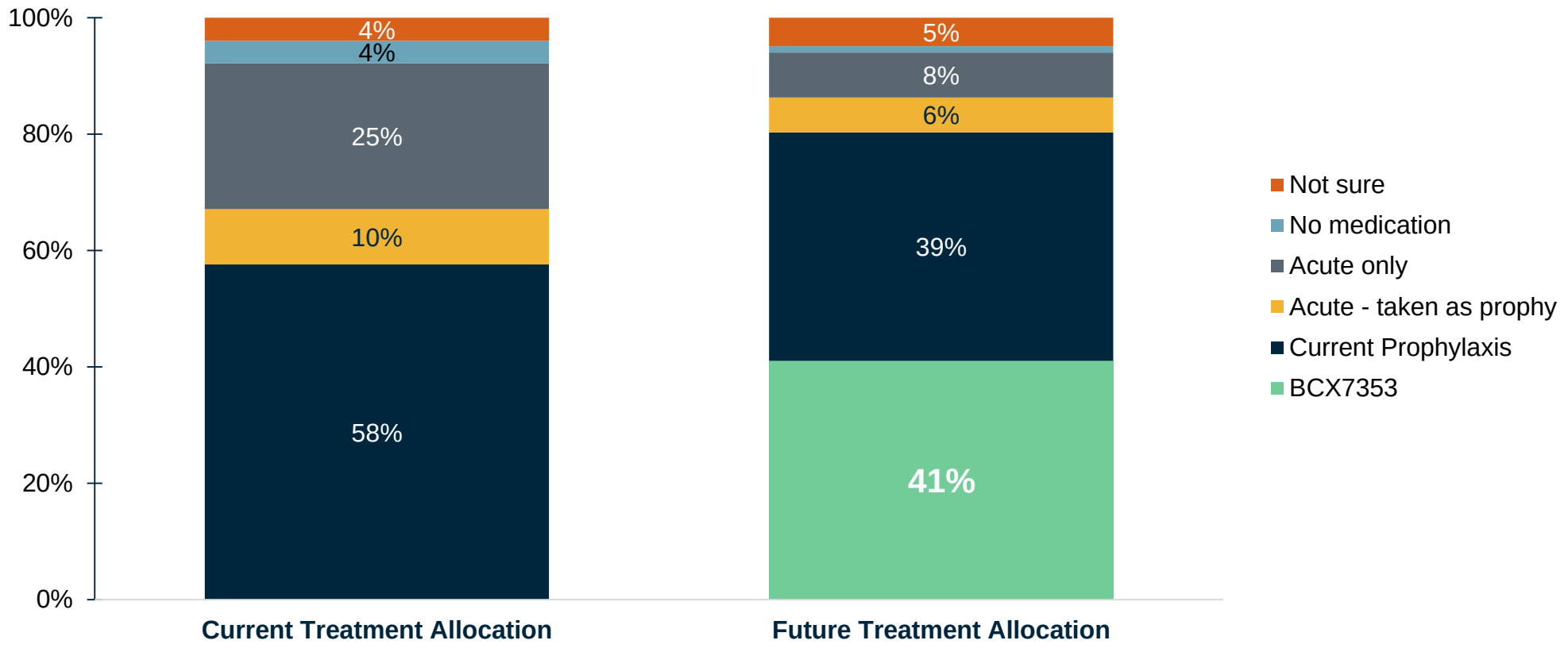
Time saving	*"Make HAE less a part of my life...less time consuming...getting on with my day. A lot of times I am so rushed and **I don't have the time to do simple things**, like do laundry or go grocery shopping. Being able to do this quickly...this is one thing that will be a lot simpler and help with my overall day."*
Less to think about and coordinate	*"**You can focus on doing other things** that are more important in life. I'd rather spend my time doing other things…going out with friends, spending time with my grandson."*
Less hassle—inconvenience	*"**Less of a hassle for me**; I work full time, I have two kids and I have a stressful, difficult life...anything I can do to prevent attacks in an easier way is less of a burden on me. Injections can be burdensome, injection site reactions, pain and swelling, dizziness."*
Less burden	*"With medications, you have to make sure that they're kept in a fridge, you have to make sure that when you take them to travel, that they don't get too hot, you have to bring ice packs, you have to bring coolers. The fact that you don't have to do that with this, again, just makes it easier. You **don't have to worry about keeping it a certain temperature."***
Not painful	*"**Less painful, not that using needles is all that painful, but it would be less painful.** Probably less work behind it. Even if it's just drawing the medication out of a vial, it's still some work that you have to do. I'd almost say that it's safer because you're not injecting something into your blood, or on your skin. You're swallowing a pill."*
Better routine	*"**Most people have a morning routine**, whether it's vitamins or taking other medication…so it would be easier than remembering every two weeks. Not worrying about the shipment, keeping it refrigerated, bent needles, the prep, etc."*

Source: BioCryst proprietary qualitative research, 2019.

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

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

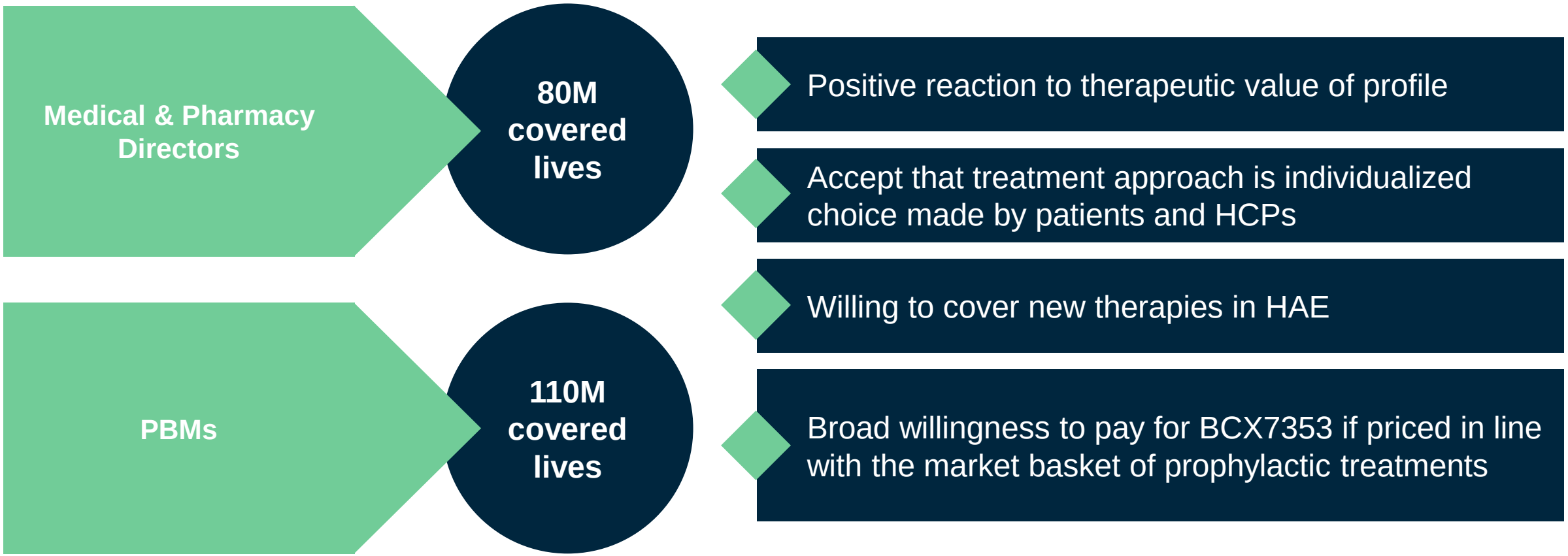
Patient Allocation by Medication Today and in Future with BCX7353 Available – Mean % of Patients



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.

Preparing for a Successful Commercial Launch

Building out critical launch elements based on our detailed market understanding
- Marketing strategy, messages and tactics
- Sales force structure and targeting
- Market access strategies

Deployed competitively-sized MSL team to call on top-tier HAE treaters

Attracting commercial leadership with extensive record of success in rare disease

Developing a best-in-class patient services and hub program

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

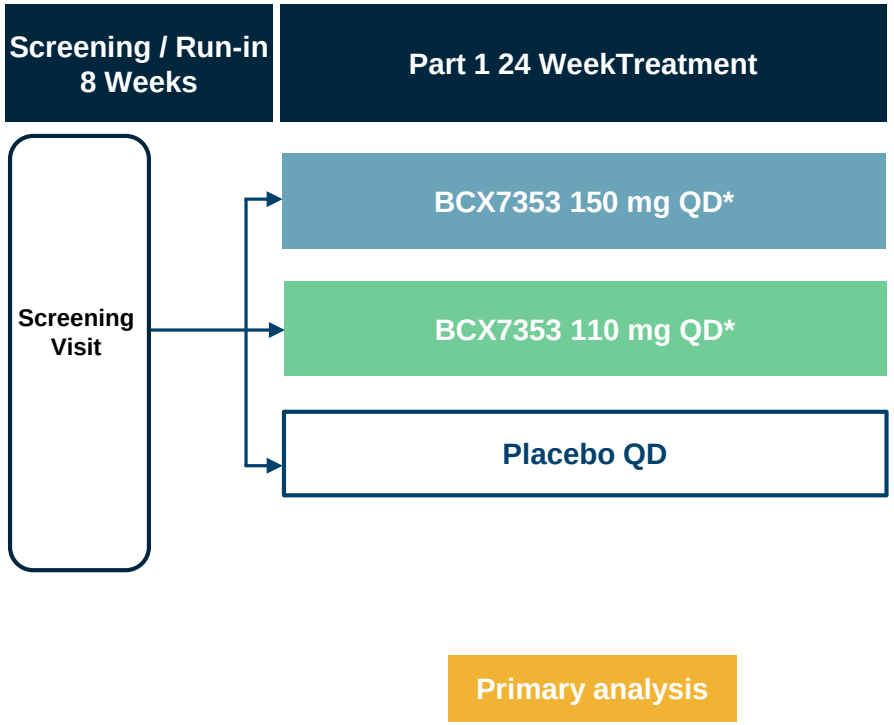
Clinical Data	Prevalence	Treatment Paradigm
Consistent, clinically meaningful benefit demonstrated through 48 weeks Safe and generally well-tolerated	~10,000 (US) HAE Patients ~7,500 diagnosed and treated	Physicians expect shift to ~80% prophylaxis

Strong Demand for Berotralstat 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)

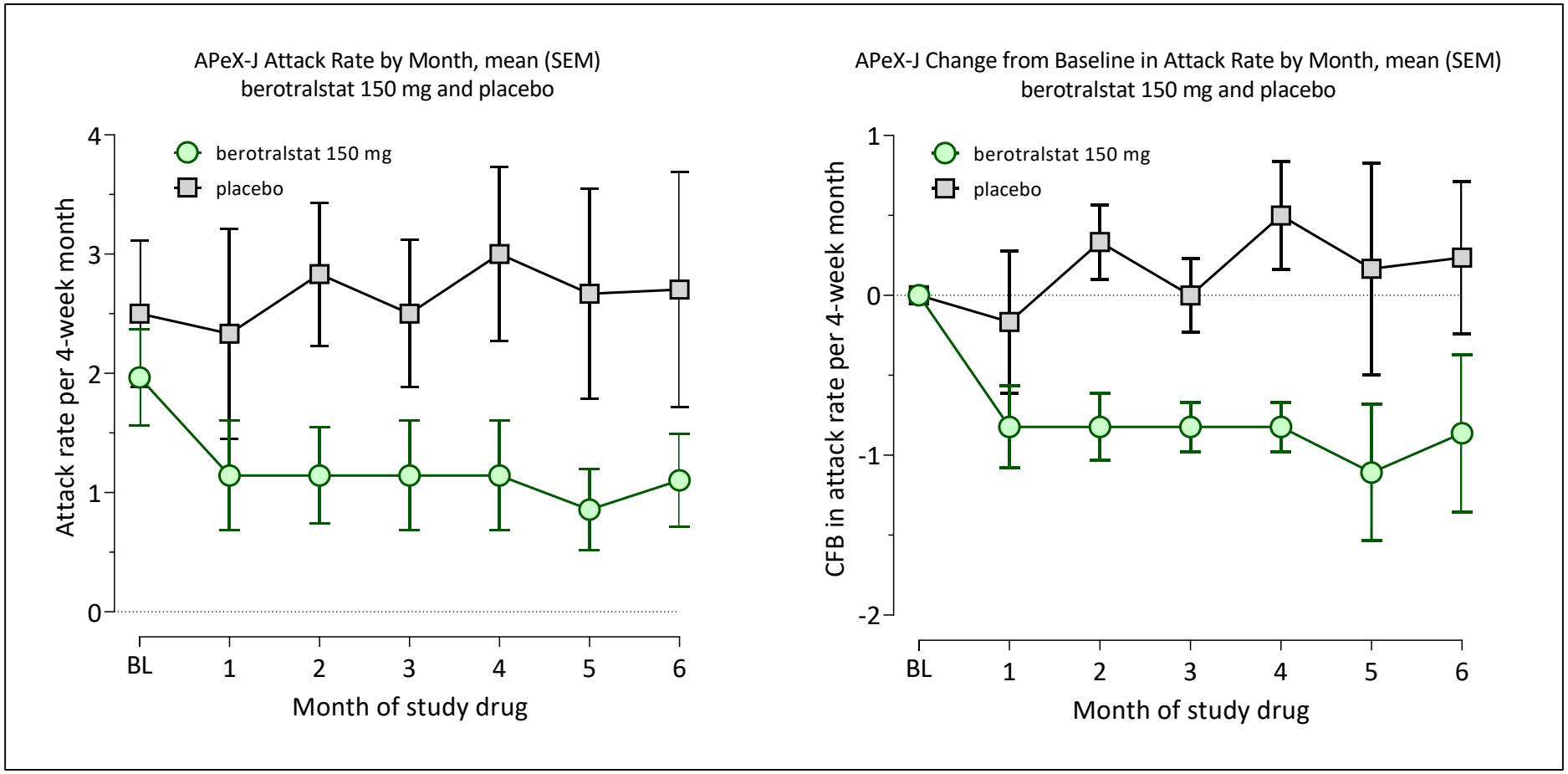


Primary endpoint: expert-confirmed angioedema attacks, rate/month*

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

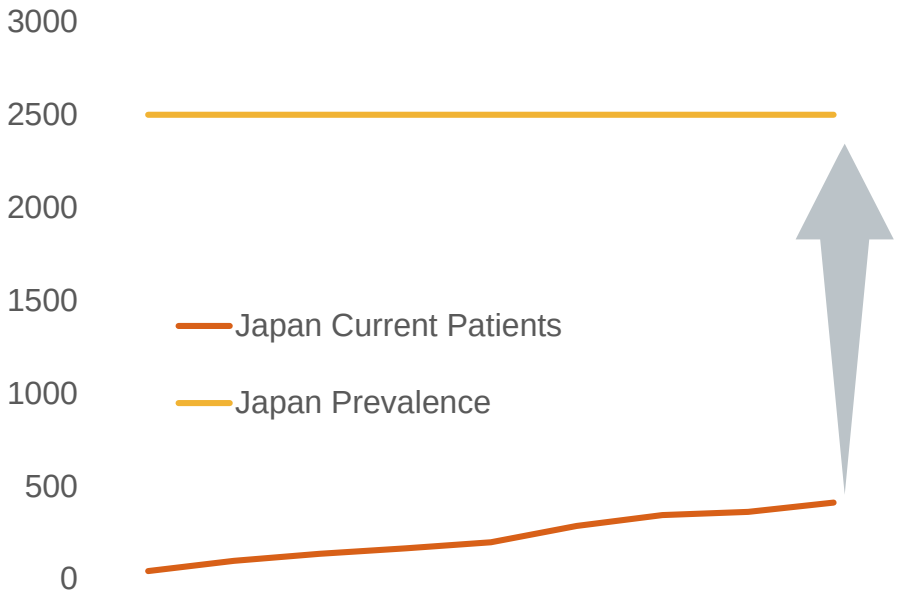


Overall Safety Summary:
Berotralstat was Safe and Generally Well Tolerated

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

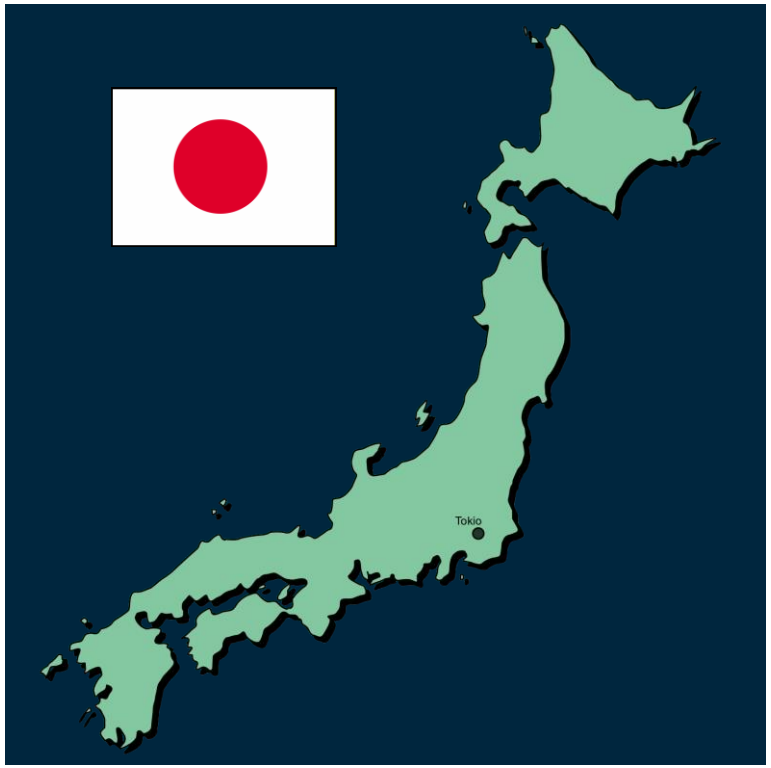
Japanese Market Growth Potential

- 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
- JNDA submitted Q1 2020
- Sakigake designation could enable Japan to be 1st global approval (2H 2020)



Clinical Update:

Dr. Bill Sheridan– Chief Medical Officer

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

Overactive AP Causes Many Complement-mediated Diseases

Seminars in Thrombosis and Hemostasis

Pathology of Renal Diseases Associated with Dysfunction of the Alternative Pathway of Complement: C3 Glomerulopathy and Atypical Hemolytic Uremic Syndrome (aHUS)

Sanjeev Sethi, MD, PhD[1] Fernando C. Fervenza, MD, PhD[2]

CJASN® Clinical Journal of American Society of Nephrology

Causes of Alternative Pathway Dysregulation in Dense Deposit Disease

Yuzhou Zhang, Nicole C. Meyer,* Kai Wang,† Carla Nishimura,* Kathy Frees,* Michael Jones,* Louis M. Katz,‡ Sanjeev Sethi,§ and Richard J.H. Smith*‖*

frontiers in Immunology

REVIEW
published: 14 June 2019
doi: 10.3389/fimmu.2019.01157

Anti-complement Treatment for Paroxysmal Nocturnal Hemoglobinuria: Time for Proximal Complement Inhibition? A Position Paper From the SAAWP of the EBMT

Antonio M. Risitano[1,2], Serena Marotta[1,2], Patrizia Ricci[1], Luana Marano[1], Camilla Frieri[1], Fabiana Cacace[1], Michela Sica[3], Austin Kulasekararaj[3,4], Rodrigo T. Calado[5], Phillip Scheinberg[6], Rosario Notaro[3†] and Regis Peffault de Latour[2,7†] on behalf of the Severe Aplastic Anemia Working Party of the European group for Bone Marrow Transplantation*

BCX9930 28-day PNH Proof of Concept Study Design

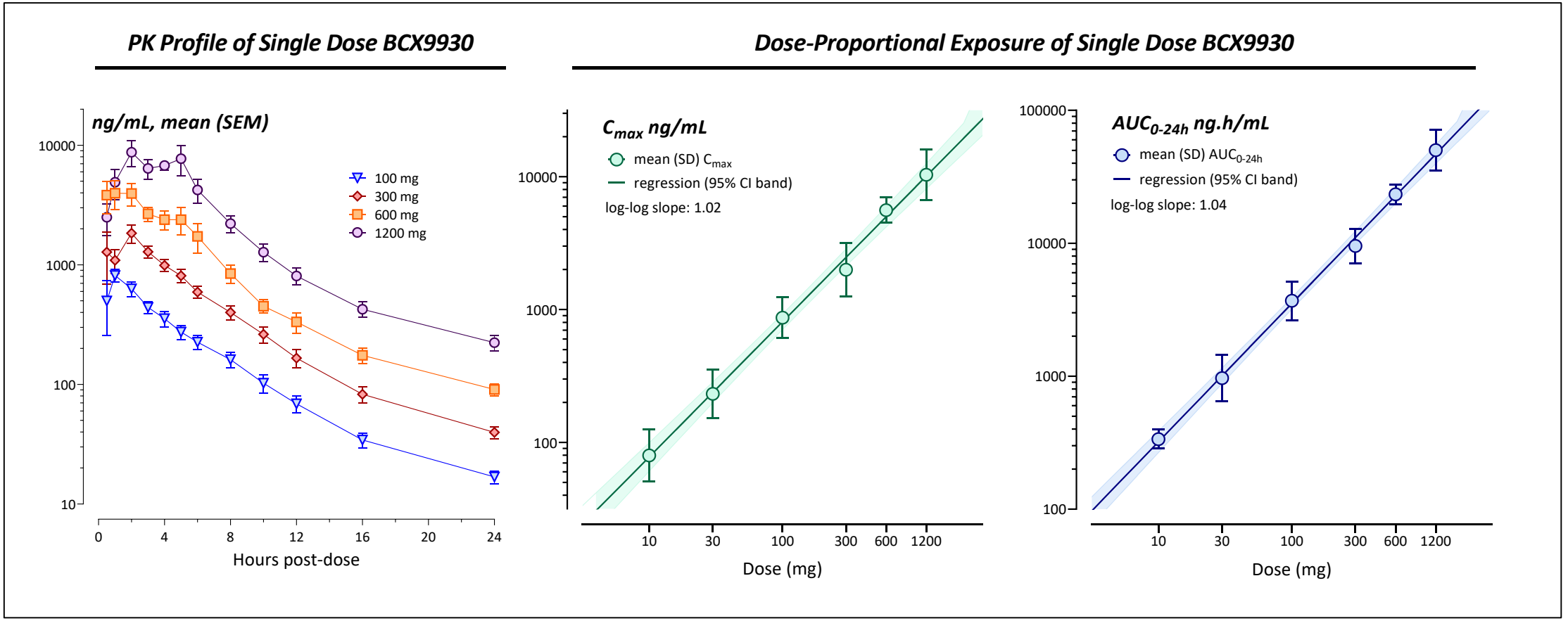
Key Outcome Measures

- **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

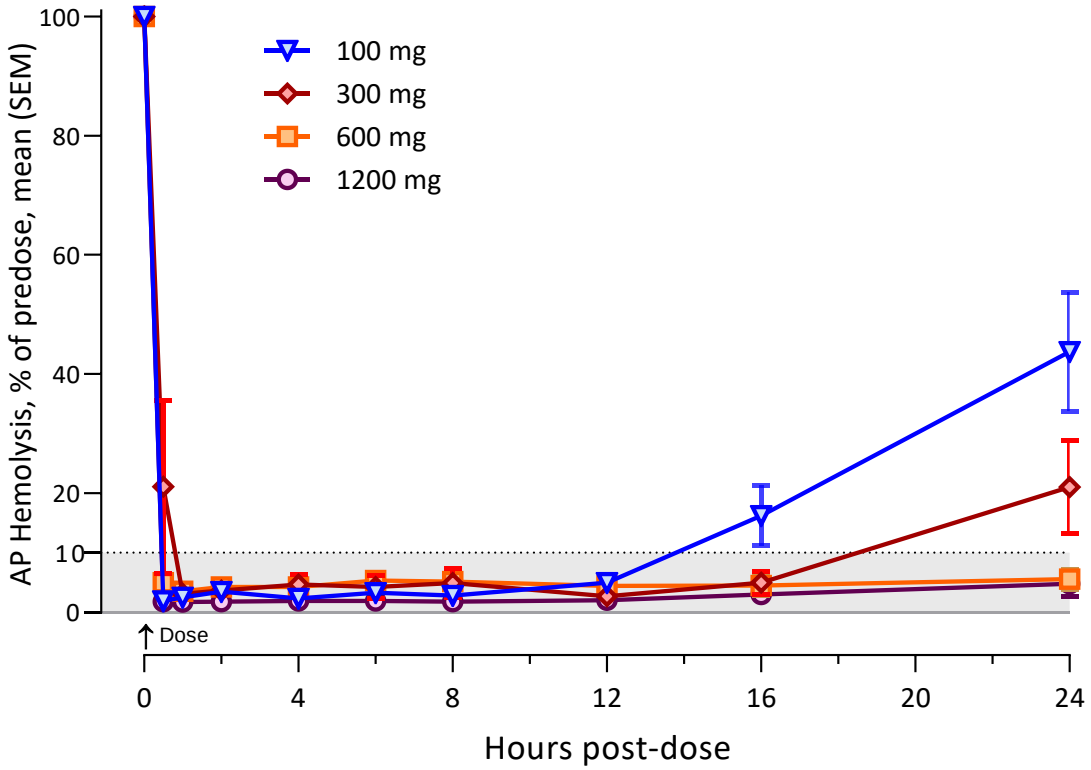
Single Dose PK Profile of Oral BCX9930 in Healthy Subjects



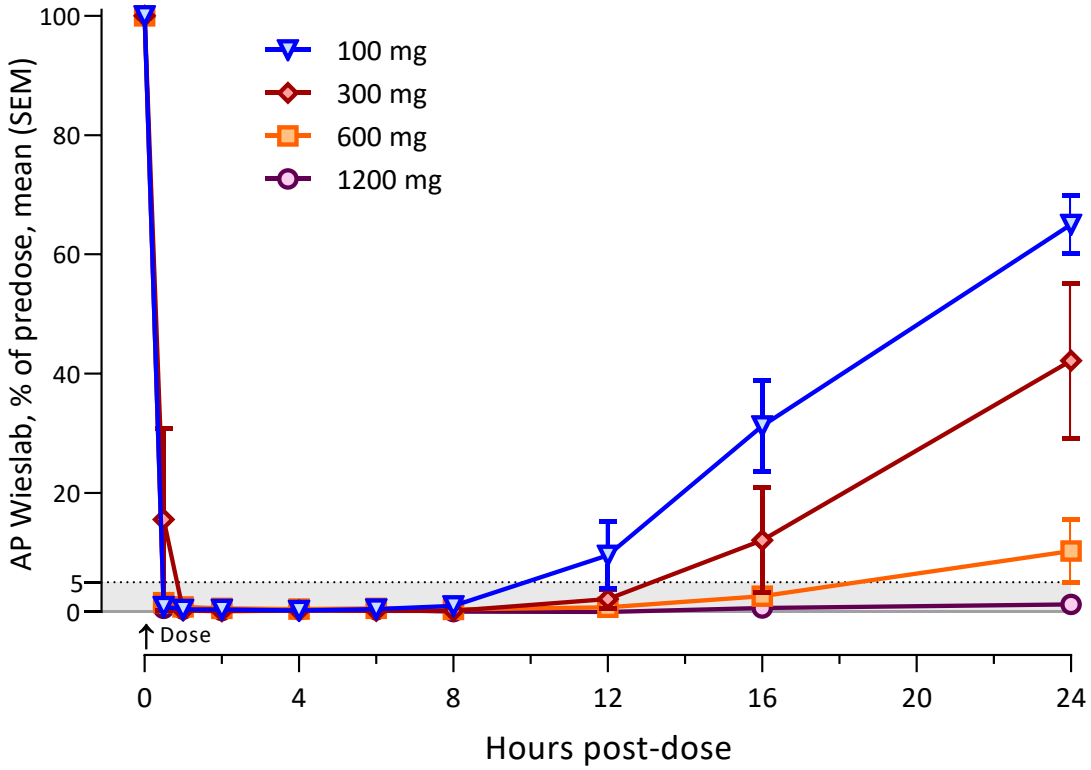
Suppression of AP Activity After Single Oral Doses of BCX9930

Alternative pathway complement activity in healthy subjects : oral BCX9930 single dose

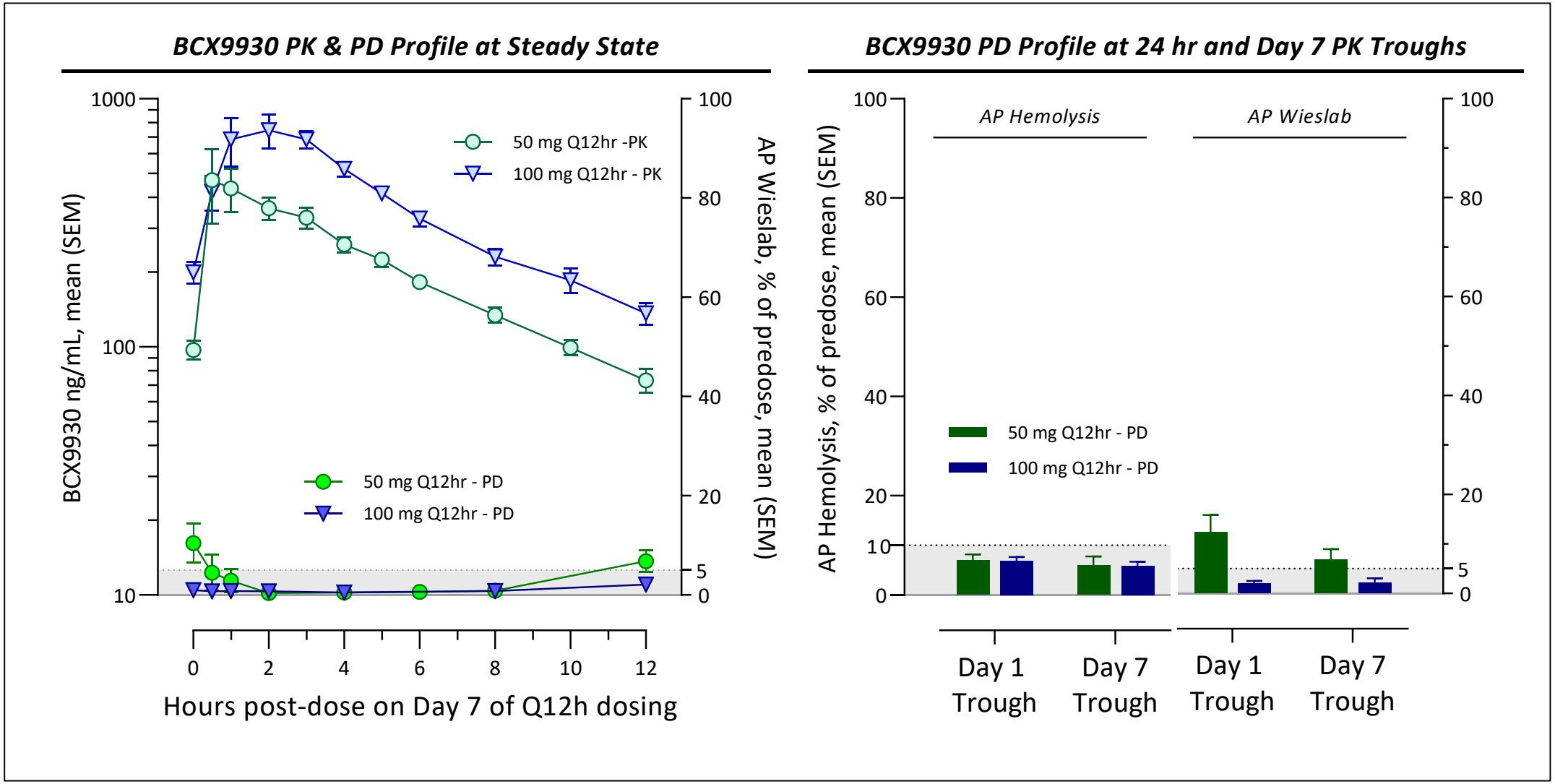
Assay: AP Hemolysis



Assay: AP Wieslab



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

Summary and Q&A

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.

Thank You…
Questions and Answers