First Quarter 2020 Results Call
Corporate Update & Financial Results

May 6, 2020

Forward-Looking Statements

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Agenda

- Corporate Update:

  Jon Stonehouse – President, Chief Executive Officer

- Global Berotralstat Launch Update:

  Charlie Gayer – Chief Commercial Officer

  Megan Sniecinski – Chief Business Officer

- Clinical Update

  Dr. Bill Sheridan – Chief Medical Officer

- Financial Update

  Anthony Doyle – Chief Financial Officer

- Summary and Q&A

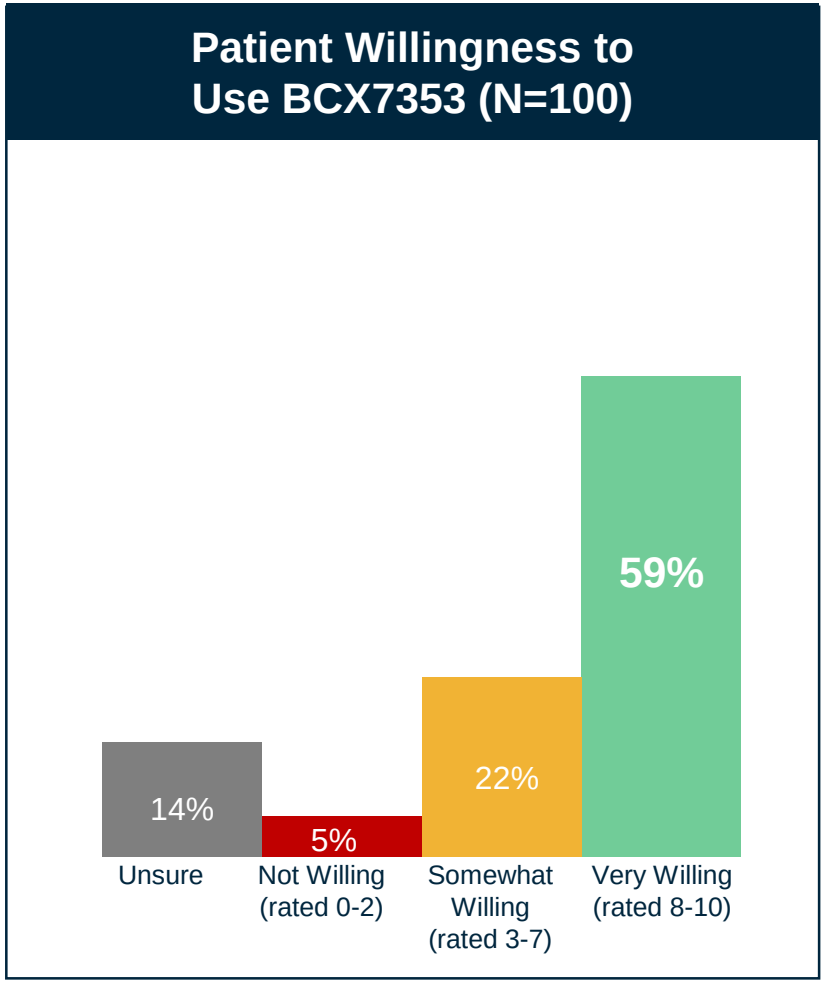
Global Berotralstat (BCX7353) Launch Update:

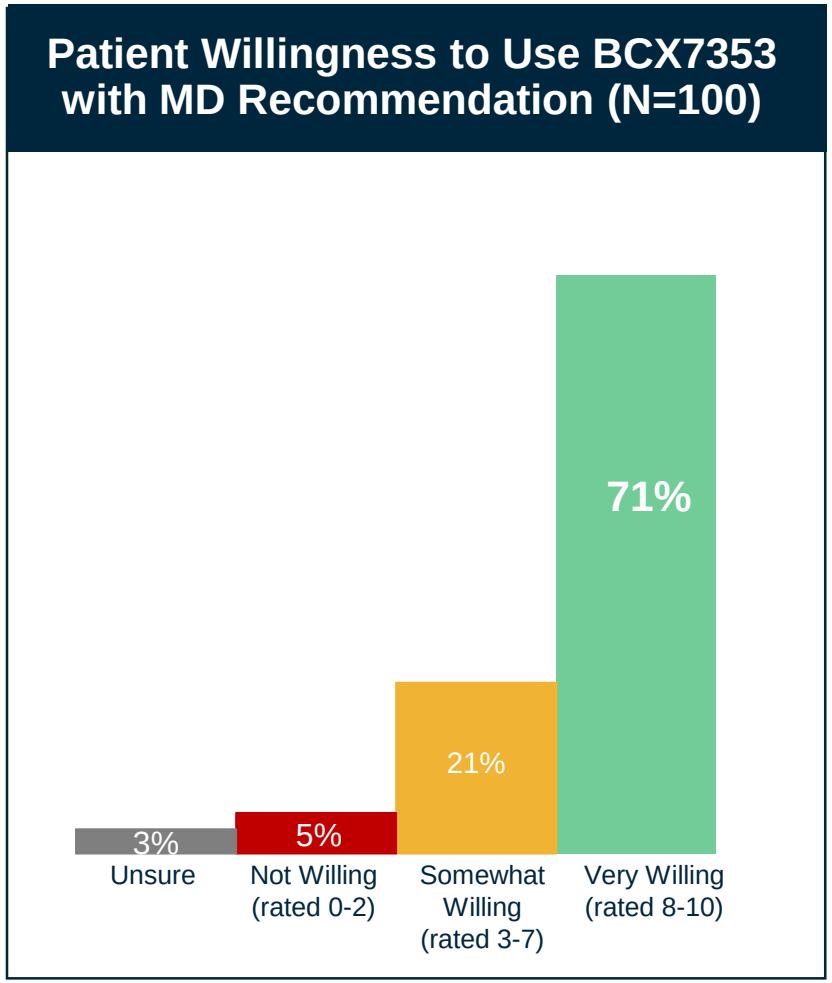
Charlie Gayer – Chief Commercial Officer

Megan Sniecinski – Chief Business Officer

Strong HAE Patient Demand for BCX7353:

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





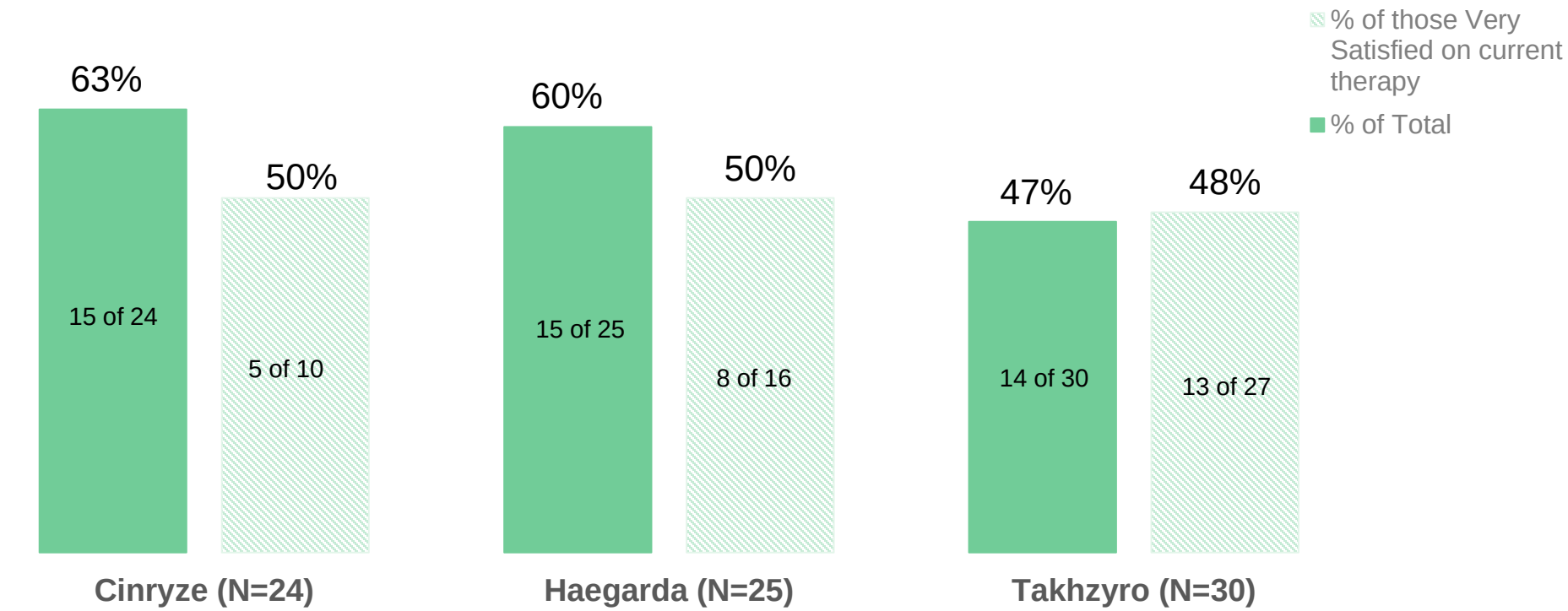
All Qualified HAE Patients (n=100)

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

Switch Opportunity—Even Those Very Satisfied with Their Current Injectable Prophylactic Treatment

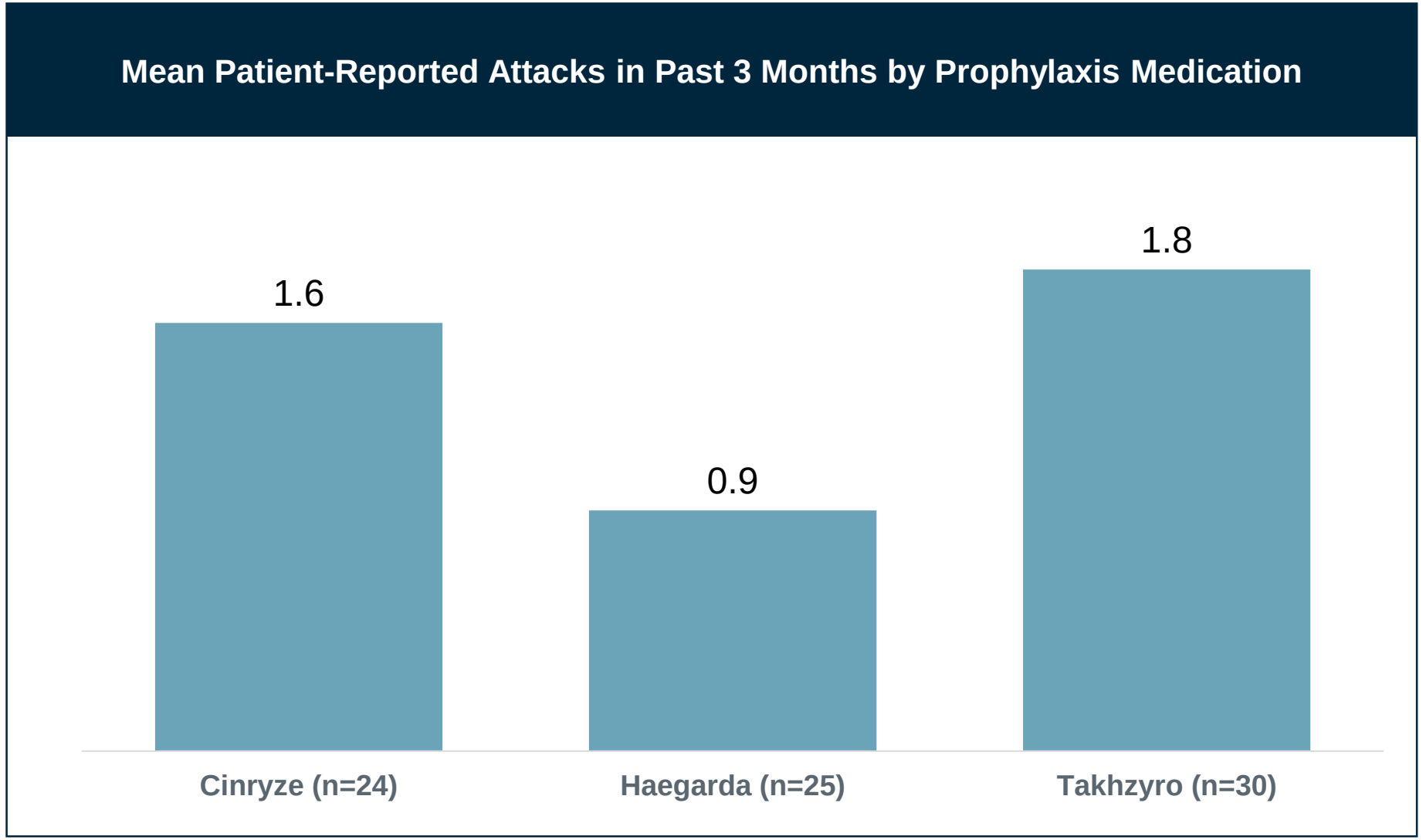
Prophylaxis Patients VERY WILLING to Use BCX7353



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



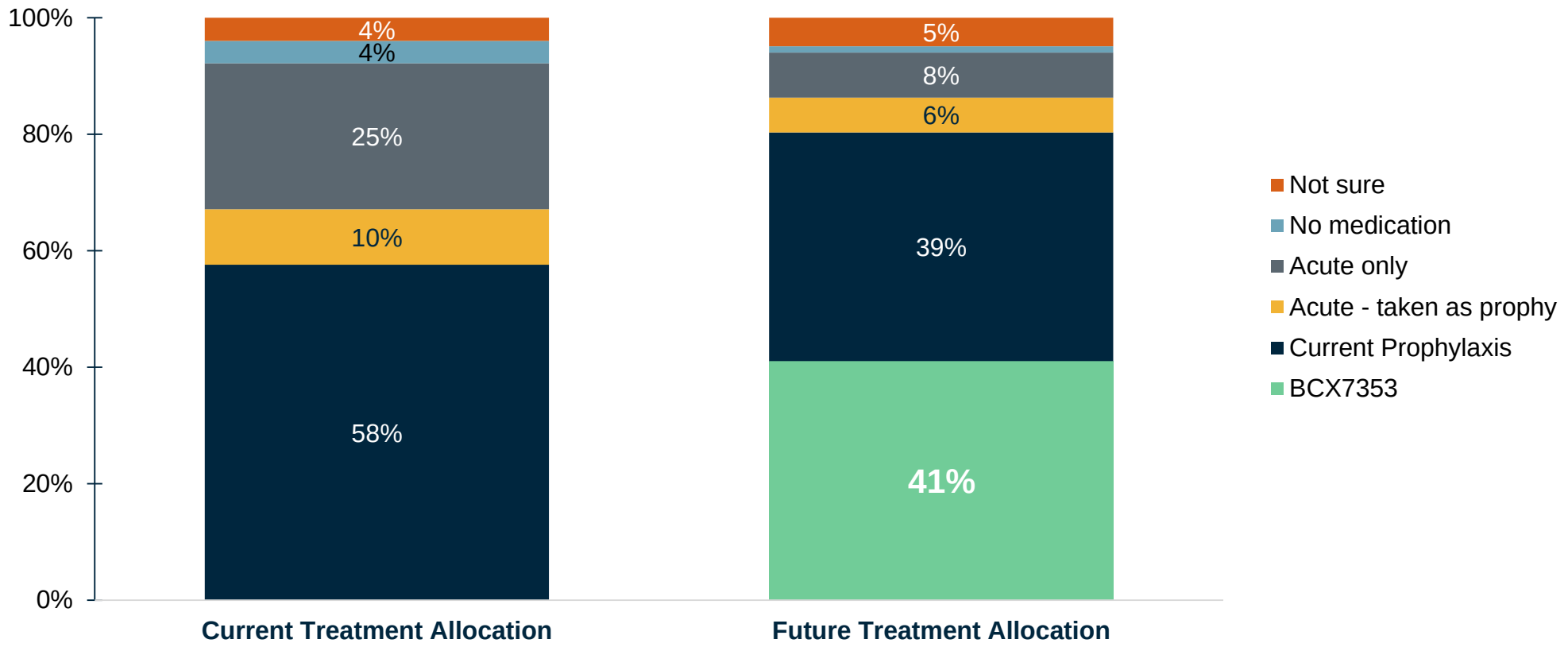
Currently Taking Medication Prophylactically

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

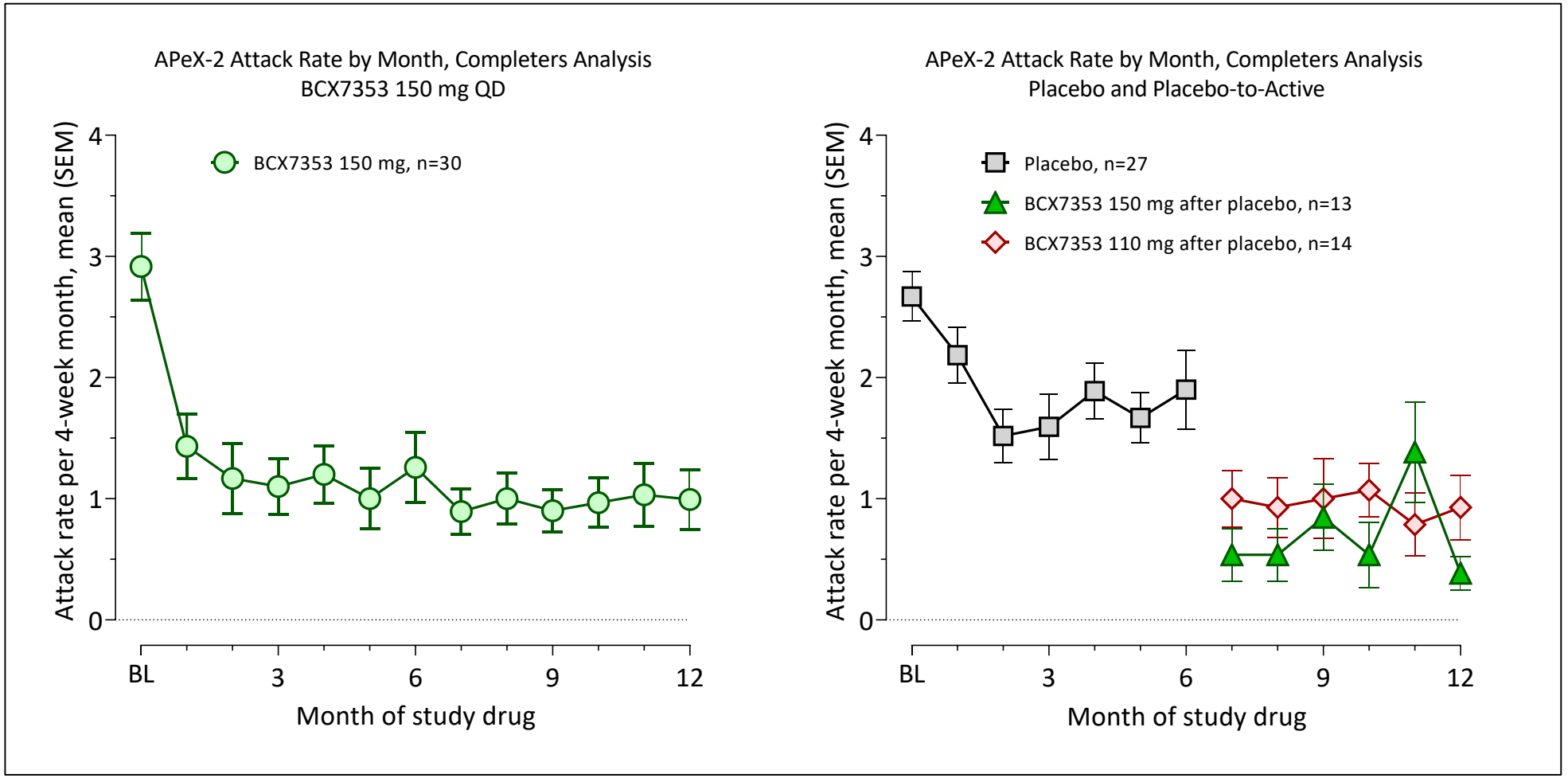
Patient Allocation by Medication Today and in Future with BCX7353 Available – Mean % of Patients



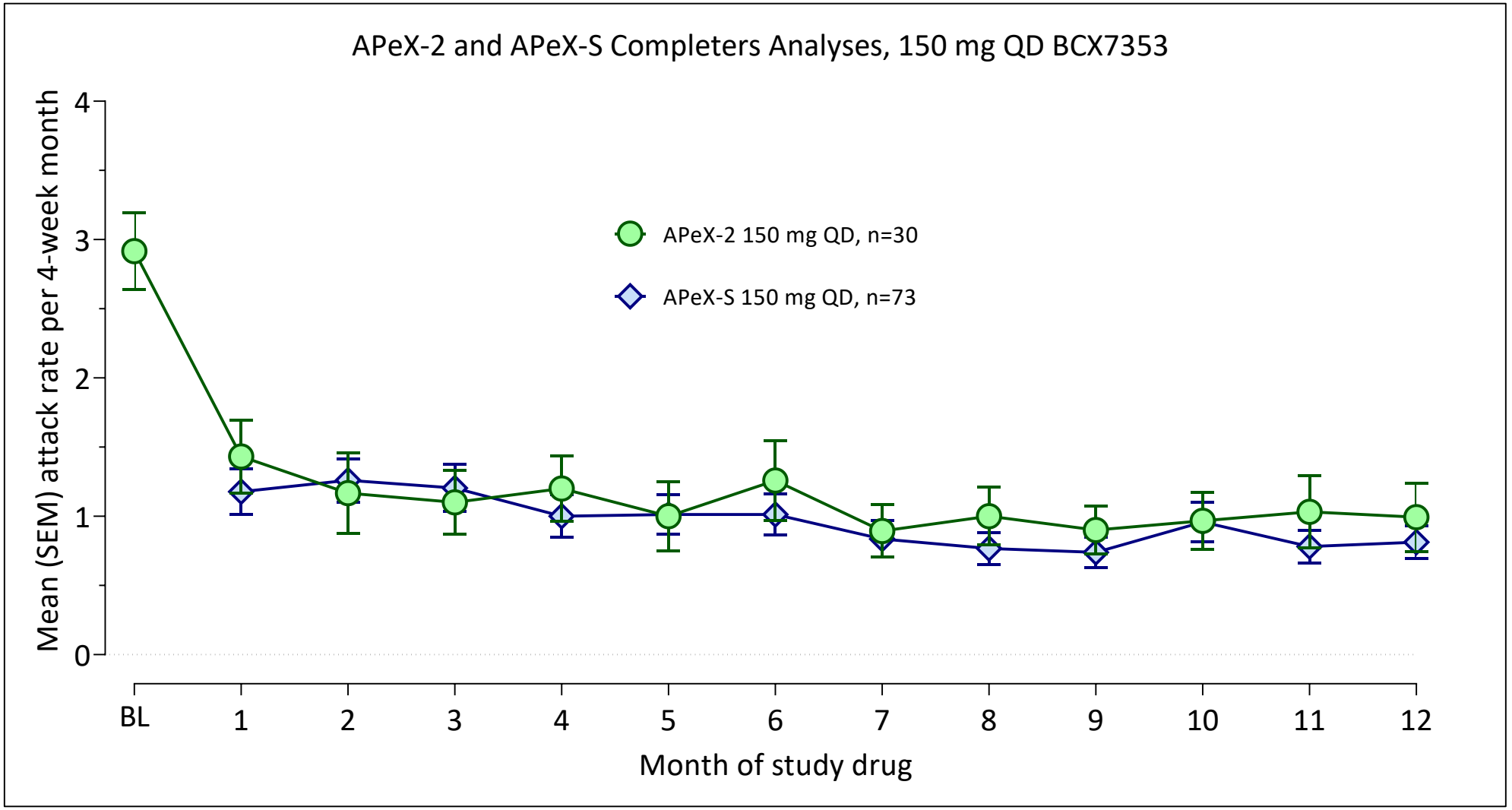
All Qualified Respondents (n=175)

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.

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

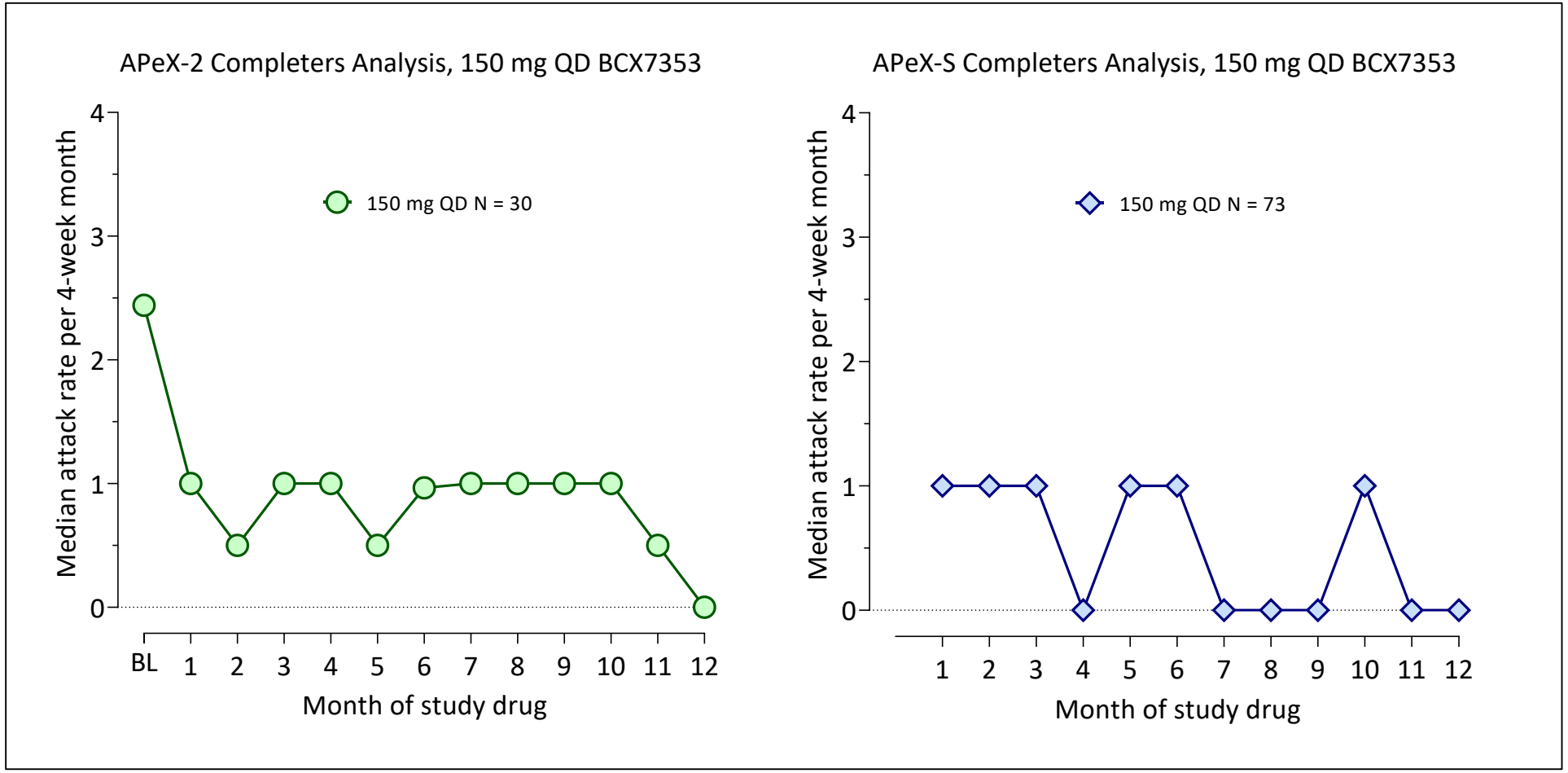


Consistent Mean Attack Rates in APeX-2 and APeX-S



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

Clinical Trial Experience Consistent with Market Research—Patients on Injectable Prophylaxis Switch to Oral Berotralstat

Physicians' expectations in market research	**~50%** of future use of berotralstat will come from patients switching from other prophylaxis treatments
APeX-2 enrollment	**44%** of patients treated previously with injected or infused C1 inhibitor prophylaxis
APeX-S enrollment in the United States	**~50%** of patients enrolled since mid-2019 previously treated with Takhzyro, Haegarda or Cinryze prophylaxis

Sources: BioCryst proprietary qualitative research, 2019; APeX-2 and APeX-S trial enrollment

Insights from Long-term Patients in APeX-2: Why they Stay on Oral, Once-Daily Berotralstat

Efficacy	***"In the past 3 months I may have had to fall back on rescue maybe 3 times, which is fantastic. I'll take that all day long. Three times in 3 months compared to twice a week [on Haegarda], this is so much better."*** ***"If I felt like a swelling going on in my stomach. Being on [berotralstat] never allowed that swelling to really run its course. I was able to eat and sleep and exercise normally… [without berotralstat] I would have had to hit pause for about 3 days."*** ***"I started to feel like I was having less HAE attacks, but more importantly, they were less severe and would be very easily controlled with the acute medications that I took."***
Tolerability	***"I haven't really experienced any side effects. Early on it sort of wanted to bother my stomach, but not anymore because now I know [to take it with a meal]."***
Less burden and improved quality of life	***"So much freer not to have all [that medicine] in your refrigerator, in your purse, when you travel… So much easier as far as not having to schedule time to mix drug and infuse it."*** ***"I travel a lot for work…[berotralstat] gave me an opportunity to never miss a treatment. It was critical in doing that. If I'd had to carry around a needle or a shot it would have been a very different process to have managed."*** ***"After several years of being a pincushion it was nice to be able to take a pill"*** ***"It was just exciting to see the difference the medication was making… All my hopes and dreams for what I was praying for started to come true, everything started to happen the way I was hoping."*** ***"You don't even realize how hard [treating HAE] is on you right now, 'cause this is all you've ever known. So I can't wait. As soon as this gets FDA approved… I'm on a bunch of patient education groups for HAE, and I've had to stay quiet about how good this works."***

Source: BioCryst proprietary qualitative research with 20 patients in APeX-2, February-March, 2020.

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

Medical Affairs team deployed and engaging with the KOL community

Experienced U.S. and EU commercial leadership team in place

Robust dual-source supply chain to support commercial launch

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

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

Targeting Overactive Alternative Pathway Could Treat Many Complement-mediated Diseases

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]

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*‖*

REVIEW
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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 PNH 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

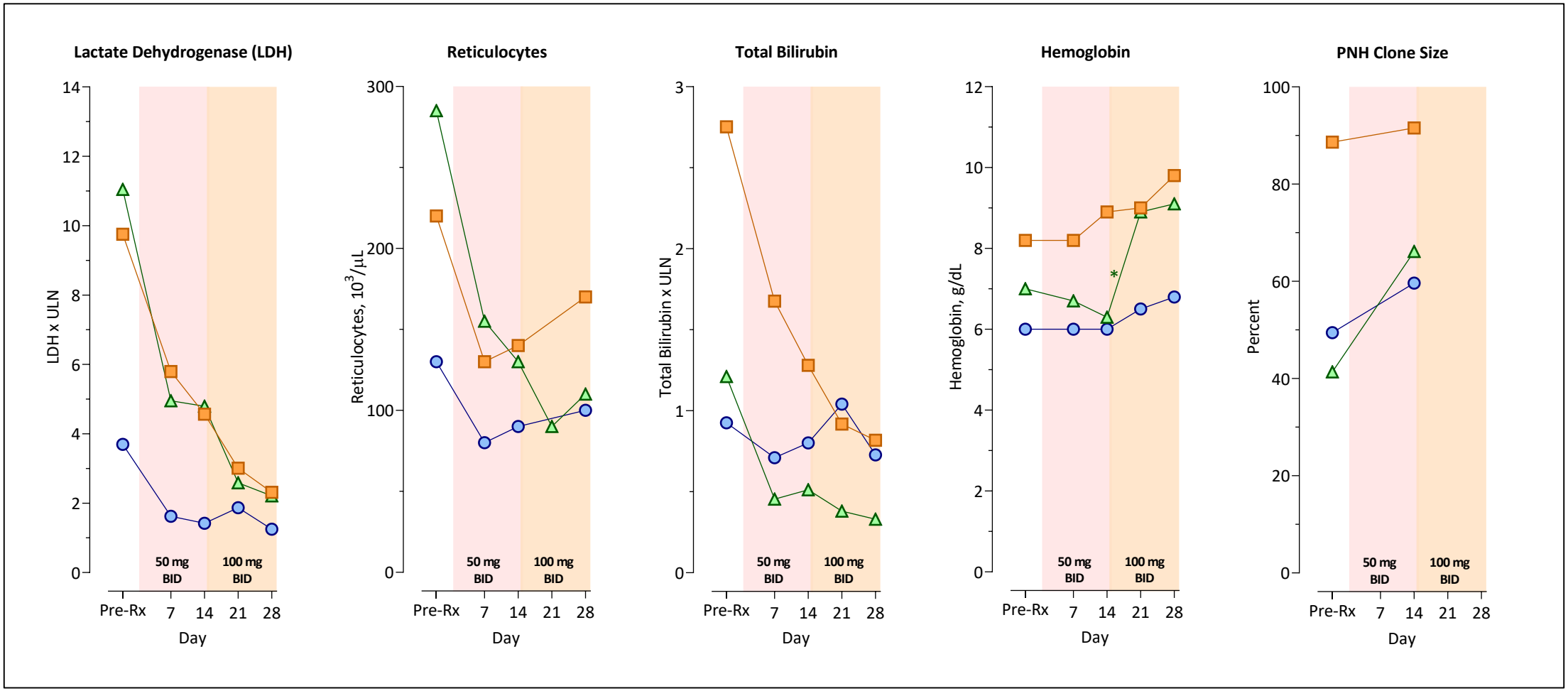
** Cohort 1 enrolment for subjects naïve to C5-INH is completed*

Treatment-naïve PNH Patients had Severe Disease

Pre-treatment Characteristics	Subject 1	Subject 2	Subject 3
PNH duration, years	8	4	3
History of aplastic anemia	no	no	yes
History of thrombosis	yes	no	no
LDH, IU/L	2205	2497	835
LDH × ULN	*9.8*	*11.0*	*3.7*
Hemoglobin, g/dL	8.2	7.0	6.0
Reticulocytes, 10^3cells/µL	220	285	130
Total bilirubin, mg/dL	3.33	1.47	1.12
PNH type III erythrocyte clone size, %	89	41	49
Units of RBC transfused in 52 weeks prior to screening	0	13	0
Units of RBC transfused in 12 weeks prior to screening	0	2	0

Laboratory values for LDH, reticulocyte count, total bilirubin and PNH type III erythrocyte clone size are average of available screening and baseline results.
Hemoglobin is last available value prior to Day 1 of BCX9930 administration. Study is ongoing – preliminary data.

Dose-dependent Improvement Across Key Indicators in Treatment-naïve PNH Subjects Receiving BCX9930 Monotherapy



Study is ongoing – preliminary data. Assays pending for RBC clone size on day 28.
Asterisk indicates RBC transfusion in Subject 2 on day 15

BCX9930 Data Provides Strong Support for Oral Monotherapy in PNH

Safety & Tolerability in PNH, n=3

- BCX9930 has been safe and generally well-tolerated in cohort 1 at low doses of 50 mg bid days 1-14 followed by 100 mg bid days 15-28
- No BCX9930-related serious adverse events
- No safety signals in routine monitoring of:
  - Vital signs, ECGs, or laboratory evaluations of hematology, coagulation, urinalysis, or clinical chemistry
- One death unrelated to study drug following 28 day study period
- 3/3 subjects had moderate headache resolving in <1-3 days soon after starting study drug
- No rash observed

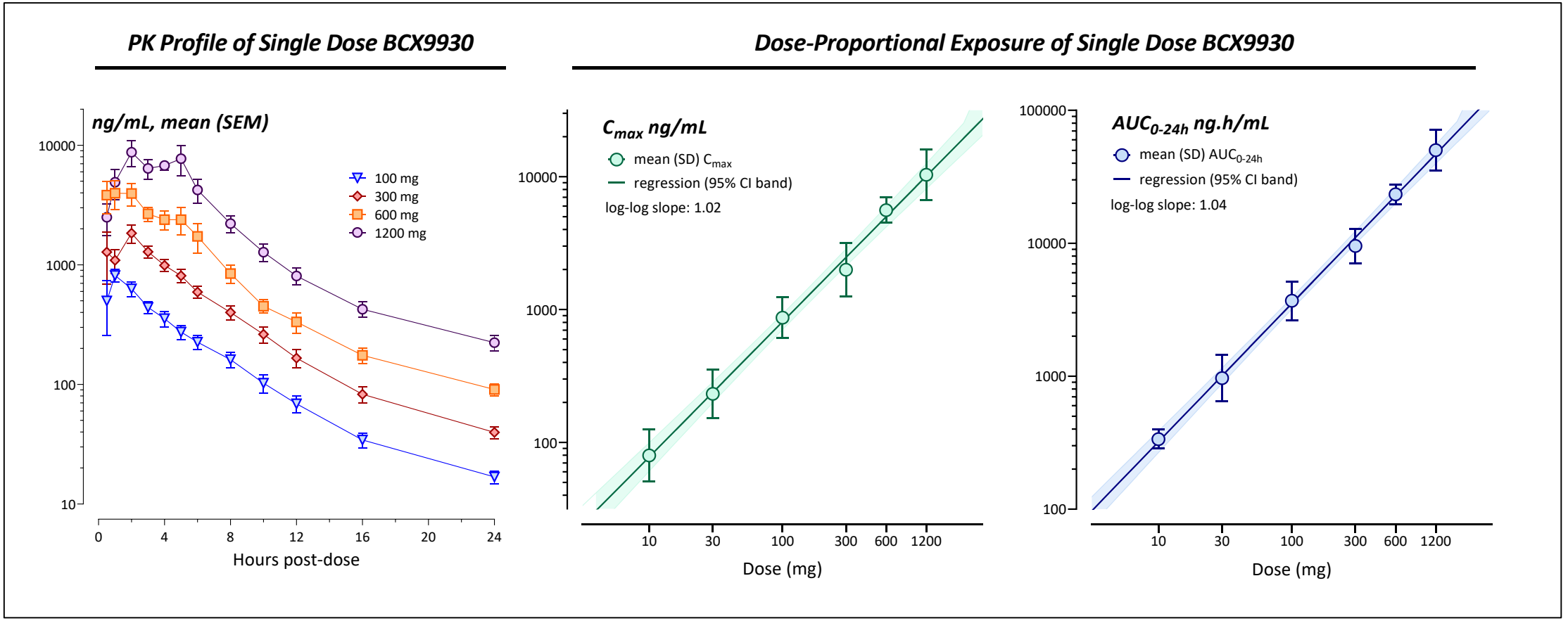
Activity in PNH at low doses, n=3

- Prompt and sustained reductions in LDH (3/3) and reticulocytes (2/3)
- Increasing PNH clone size and Hb
- Investigator assessed clinical benefit in 3/3 patients, all continued to long-term extension

Next steps

- Open 200/400 mg bid cohort for C5-inhibitor naïve patients after completing cohort 1, data expected Q3 2020
- Enroll C5-inhibitor poor responders in 200/400 mg bid cohort in Q3 2020, data expected by YE 2020

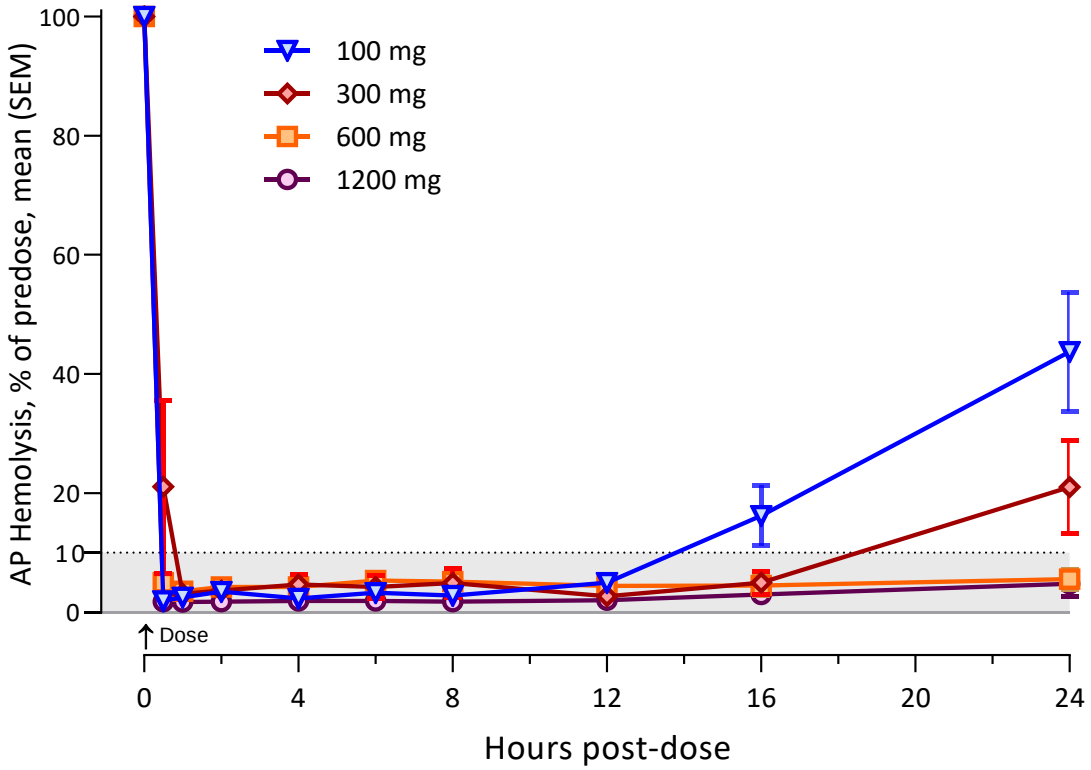
Single Dose PK Profile of Oral BCX9930 in Healthy Subjects



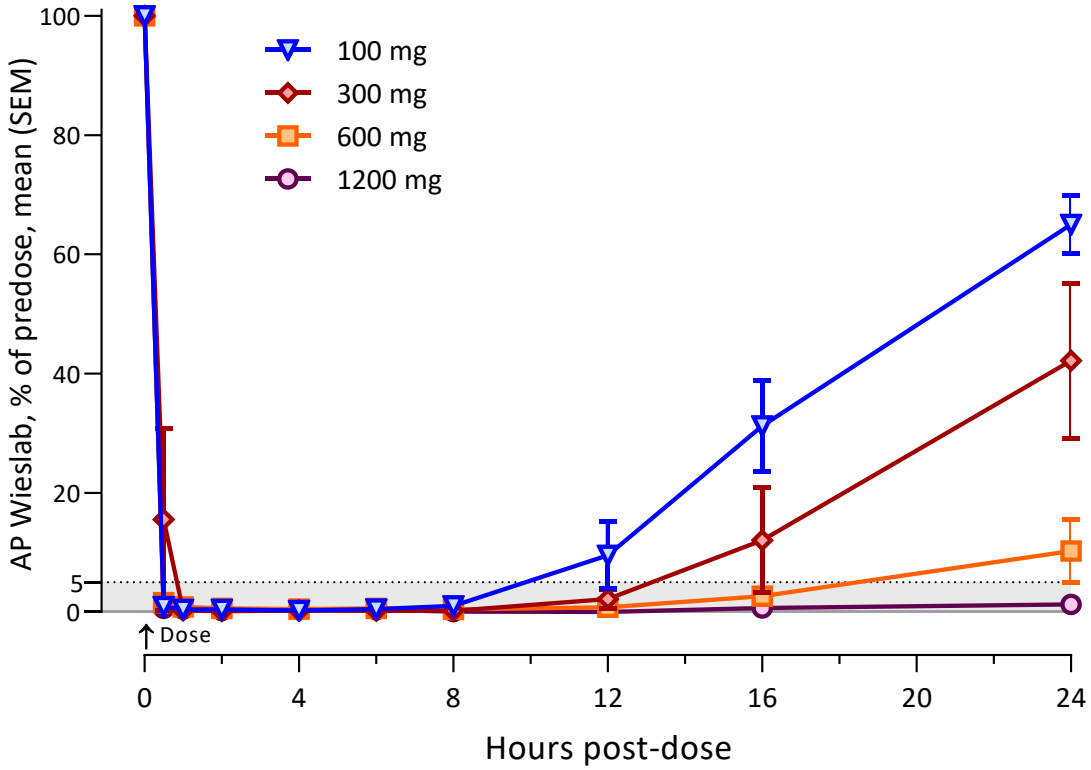
Suppression of AP Activity After Single Oral Doses of BCX9930

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

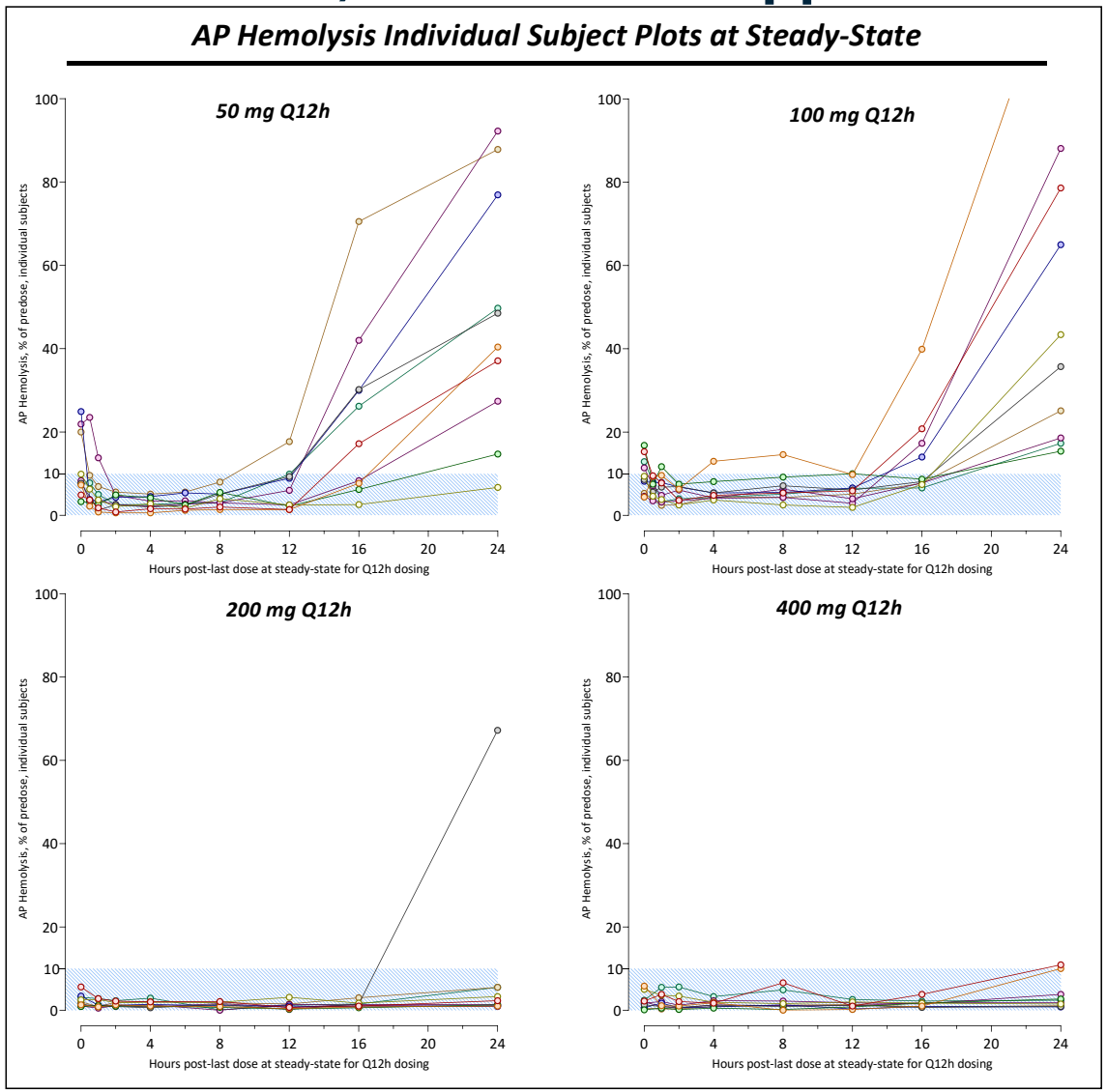
Assay: AP Hemolysis

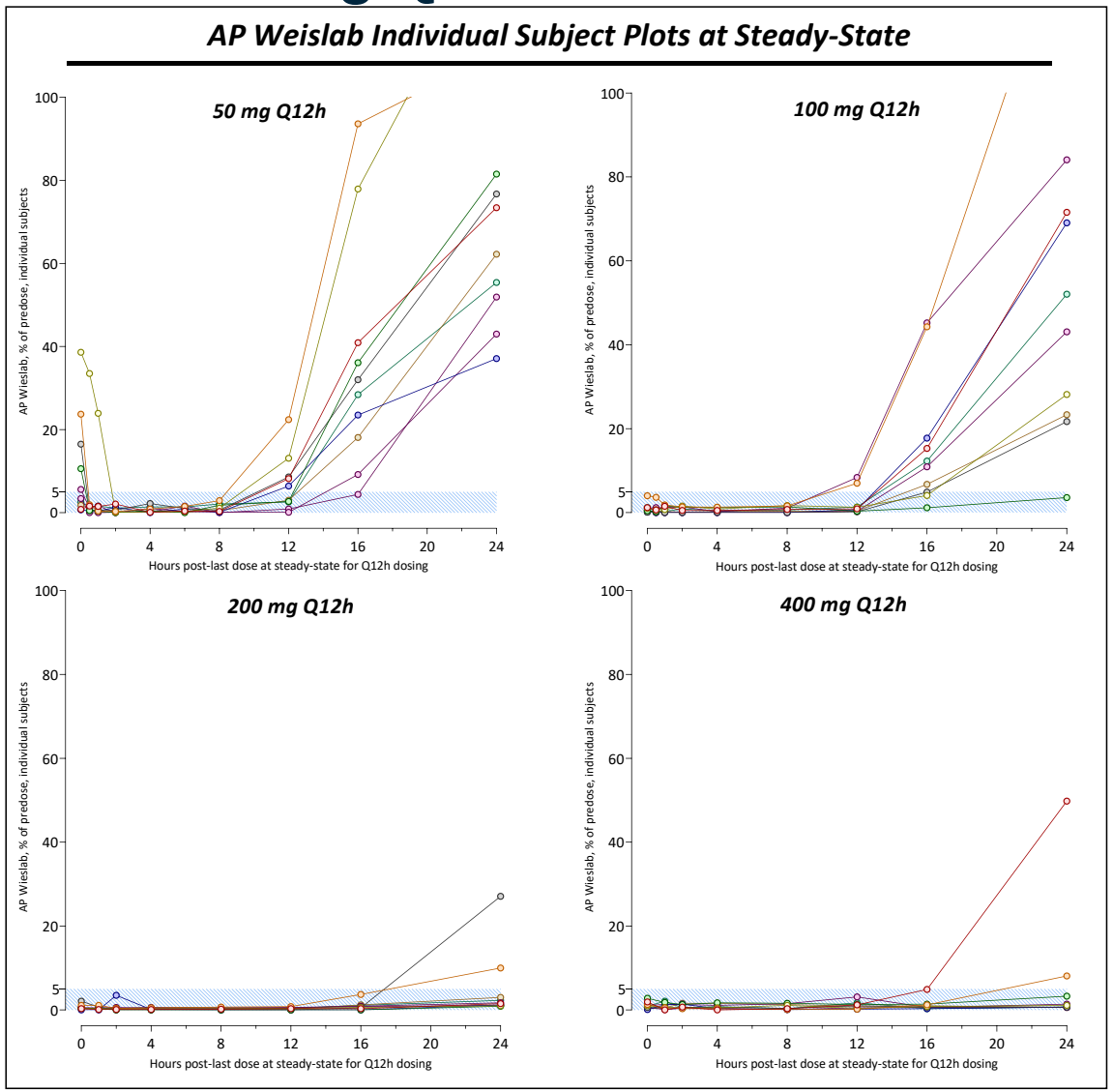


Assay: AP Wieslab



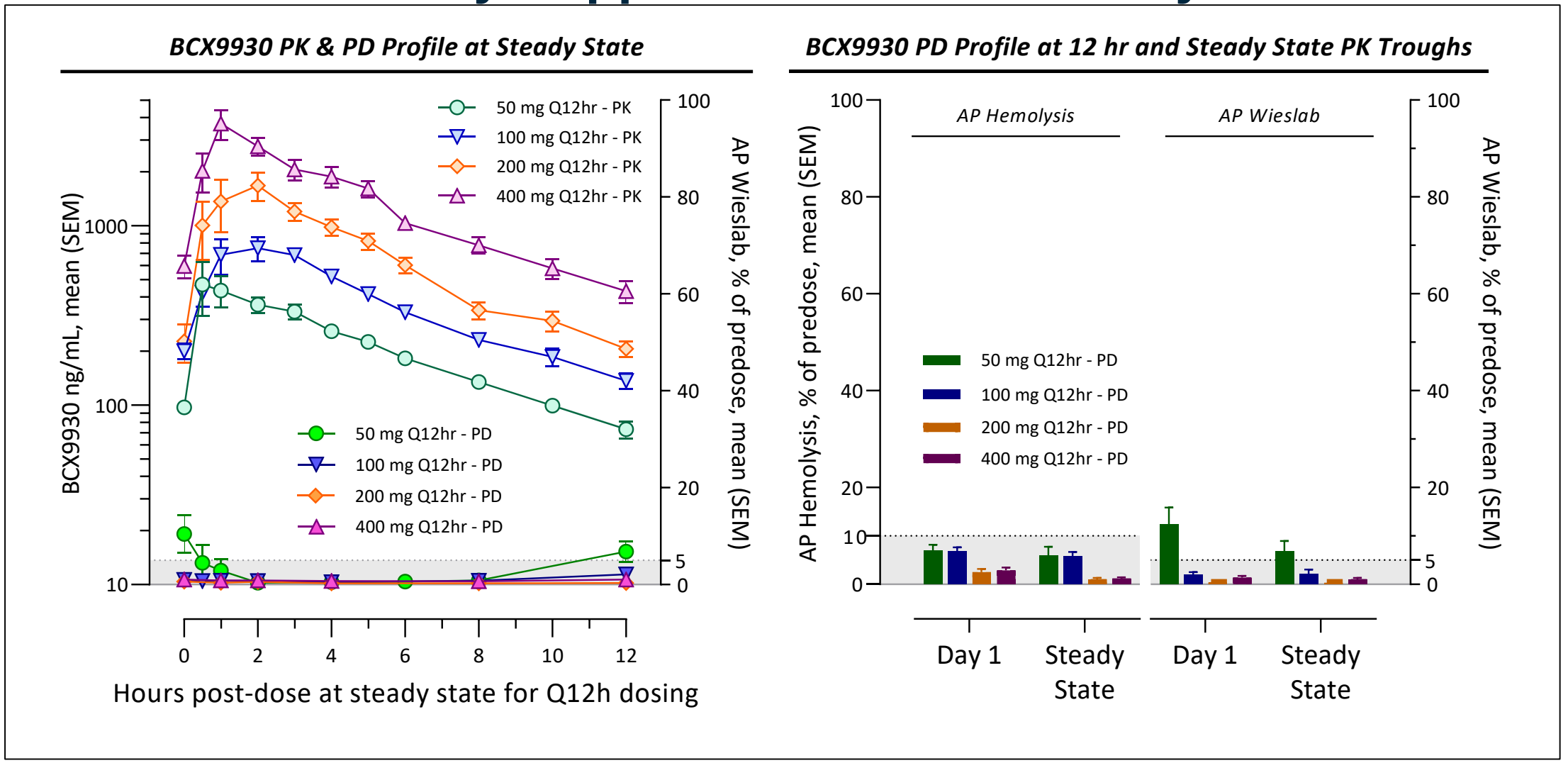
Clear Dose-response in AP Inhibition – Consistent, Sustained Suppression at 200/400 mg Q12h





Last dose of BCX9930 on Q12 hr schedule was administered at time 0, and results through 24 hours post-dose are shown

Greater Exposure at 200/400 mg with >98% Sustained Alternative Pathway Suppression in Both Assays



Successful BCX9930 SAD/MAD Supports Monotherapy for Diseases of the Alternative Pathway

Safety & Tolerability: Healthy Subjects

- Study drug was 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
- Benign rash in majority of MAD subjects was self-limited and resolved within a median of 5 days of onset
- No dose-related safety signals observed

PK/PD in Healthy Subjects

- Linear, dose-proportional exposure
- Dose-related suppression of AP of complement functional activity
- > 98% inhibition of AP in both AP Wieslab and AP hemolysis assays at steady-state dosing for doses of 200 mg Q12h and 400 mg Q12h

Next Steps

- Test supratherapeutic doses to finish SAD/MAD
- Explore once-daily dosing

Galidesivir Clinical Trial in COVID-19 Enrolling Patients

Part 1 (n=24)

Cohort 1 *GVR n=6 , PBO n=2*	10 mg/kg then 2 mg/kg q12h×13
Cohort 2 *GVR n=6 , PBO n=2*	10 mg/kg then 5 mg/kg q12h×13
Cohort 3 *GVR n=6 , PBO n=2*	20 mg/kg then 5 mg/kg q12h×13

Part 2 - Randomized 2:1 (GVR:PBO)

Cohort	Dose selected from Part 1 (n=42)

Key Outcome Measures

- Safety
- PK
- Viral Load Reduction
- Changes in clinical signs and symptoms

Cash position & 2020 guidance (in millions)

Cash & investments at December 31, 2019	$137
Cash & investments at March 31, 2020	**$115**
Senior Credit Facility	$50
FY 2020 GUIDANCE	
Net operating cash utilization	**$125 – 150**
Operating expenses [A]	**$135 – 160**

A - Excludes equity-based compensation.

Thank You…
Questions and Answers