

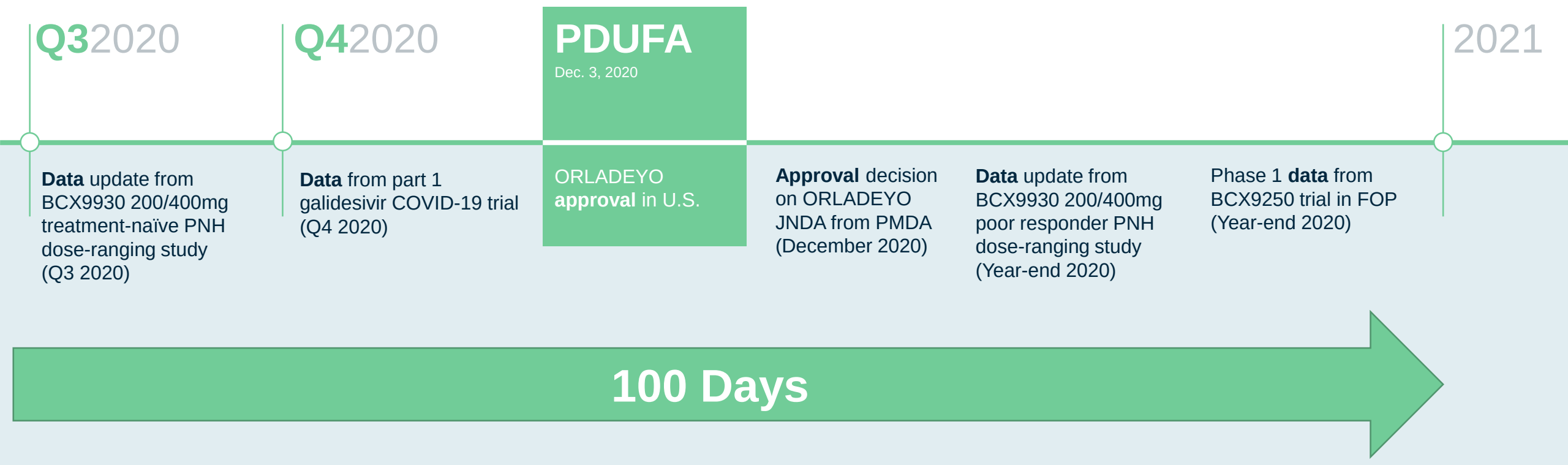
September 2020 Corporate Presentation



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Six Major Program Updates Expected in Next 100 Days



Coming Soon: Orladeyo™

Orladeyo™
(berotralstat) 150 mg capsule



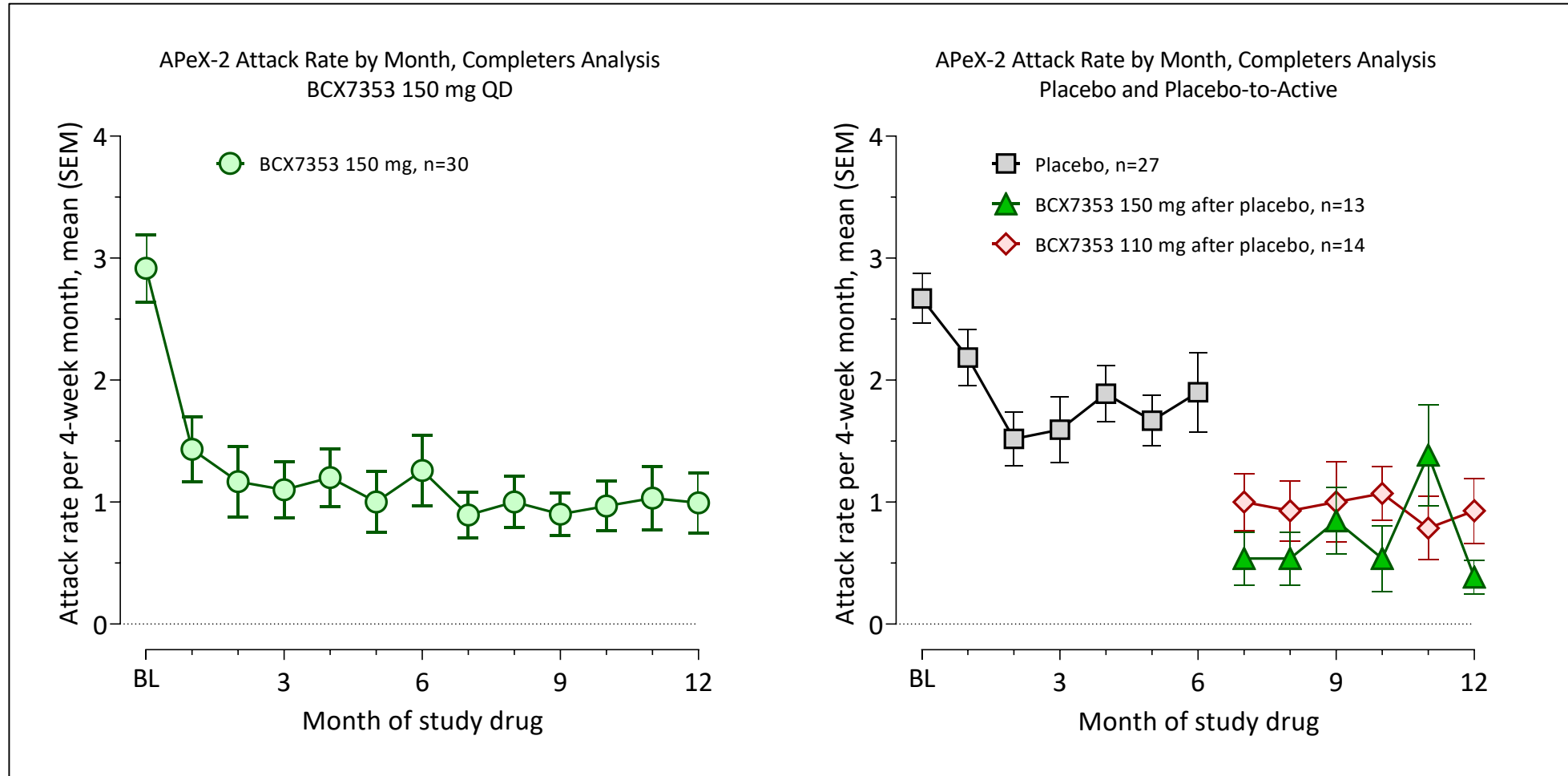
COMING SOON

Orladeyo™
(berotralstat) 150 mg capsule

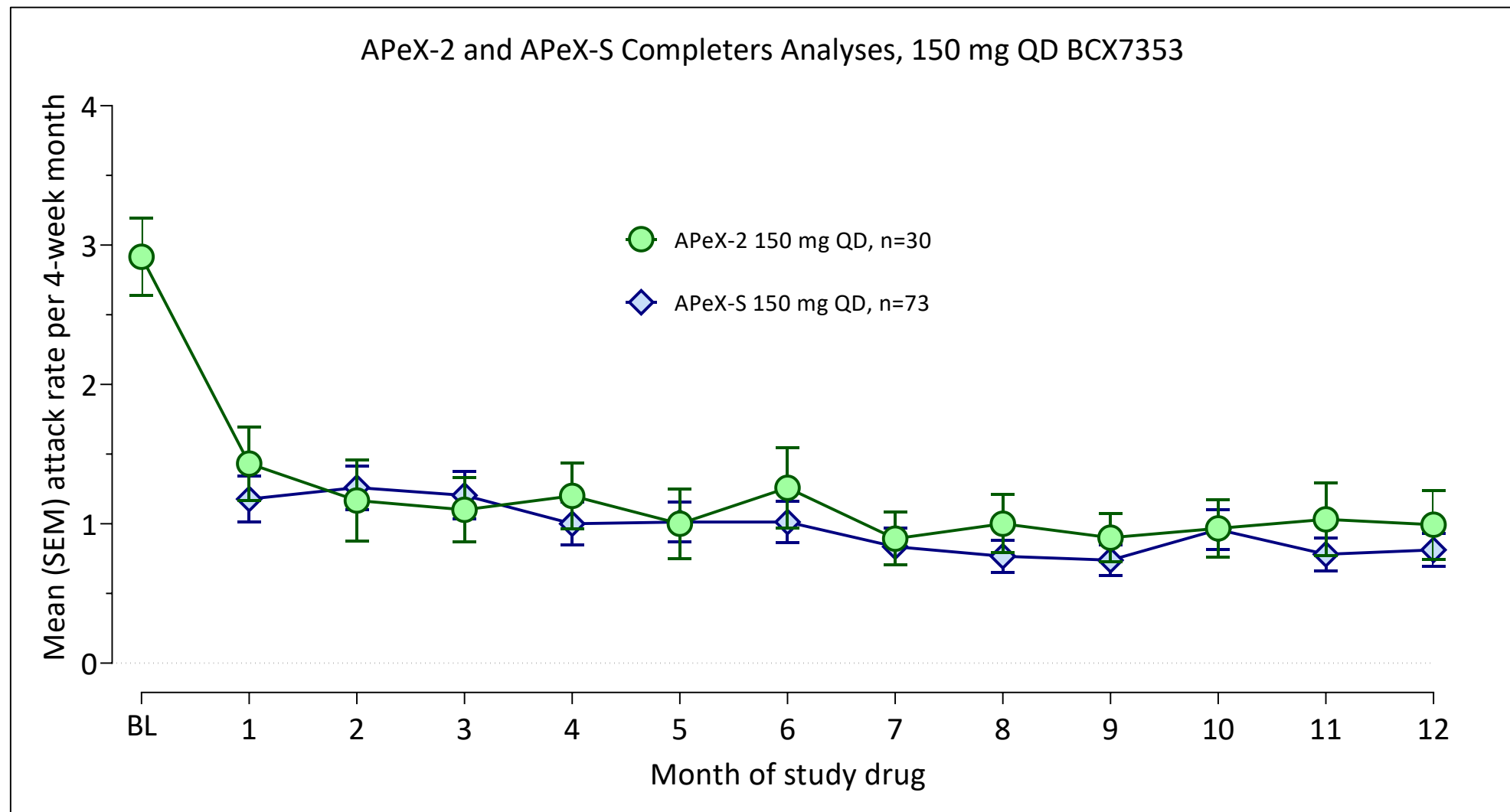
Sign up to find out first at
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Rapid and Sustained HAE Attack Rate Reduction: Analyses for APeX-2: 150 mg and Placebo 48-week Completers

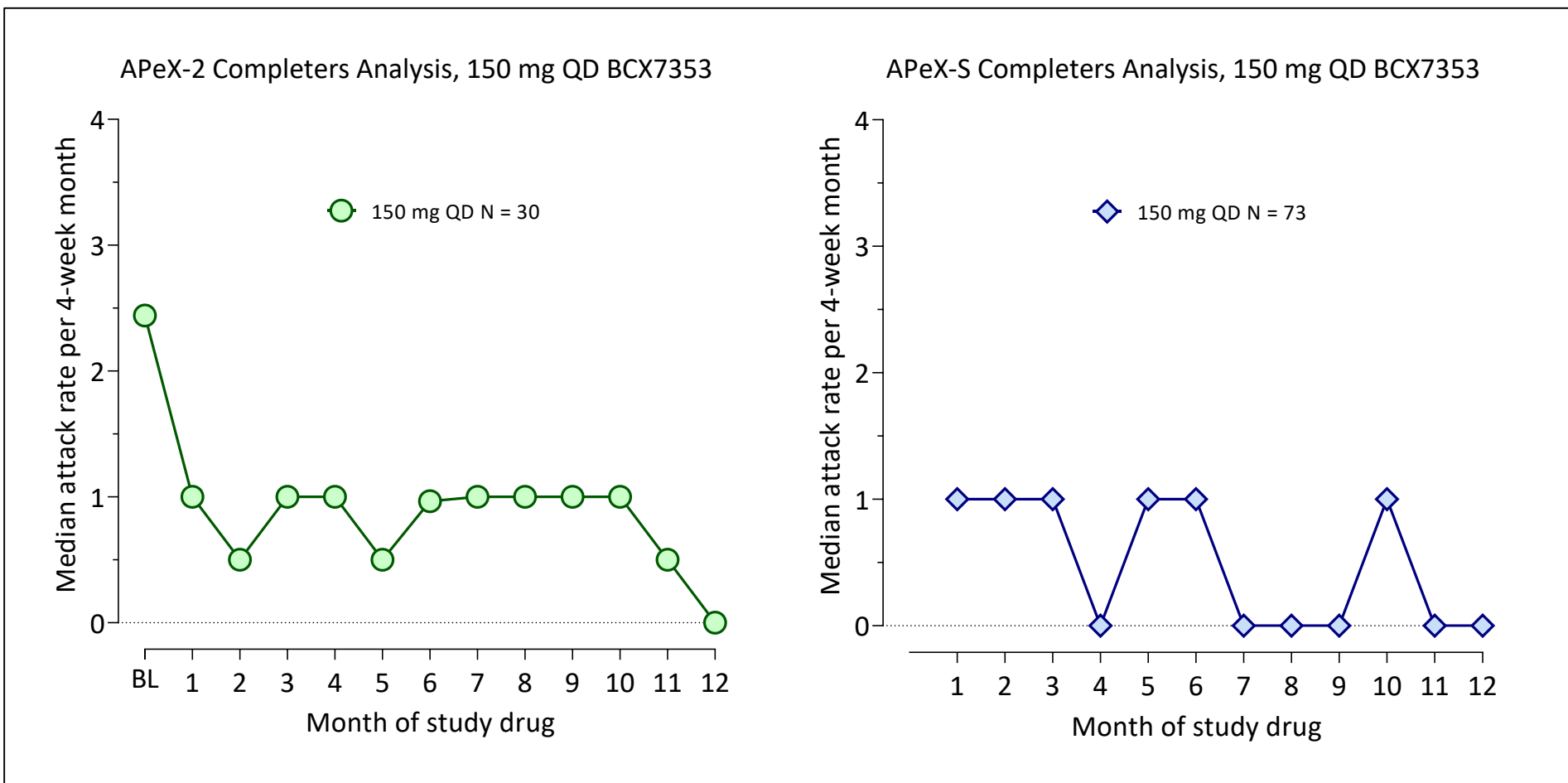


Consistent Mean Attack Rates in APeX-2 and APeX-S



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
<i>Subjects enrolled and dosed [Safety Population]</i>	<i>N = 158</i>	<i>N = 184</i>	<i>N = 39</i>
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-Related⁶			
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			

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

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

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

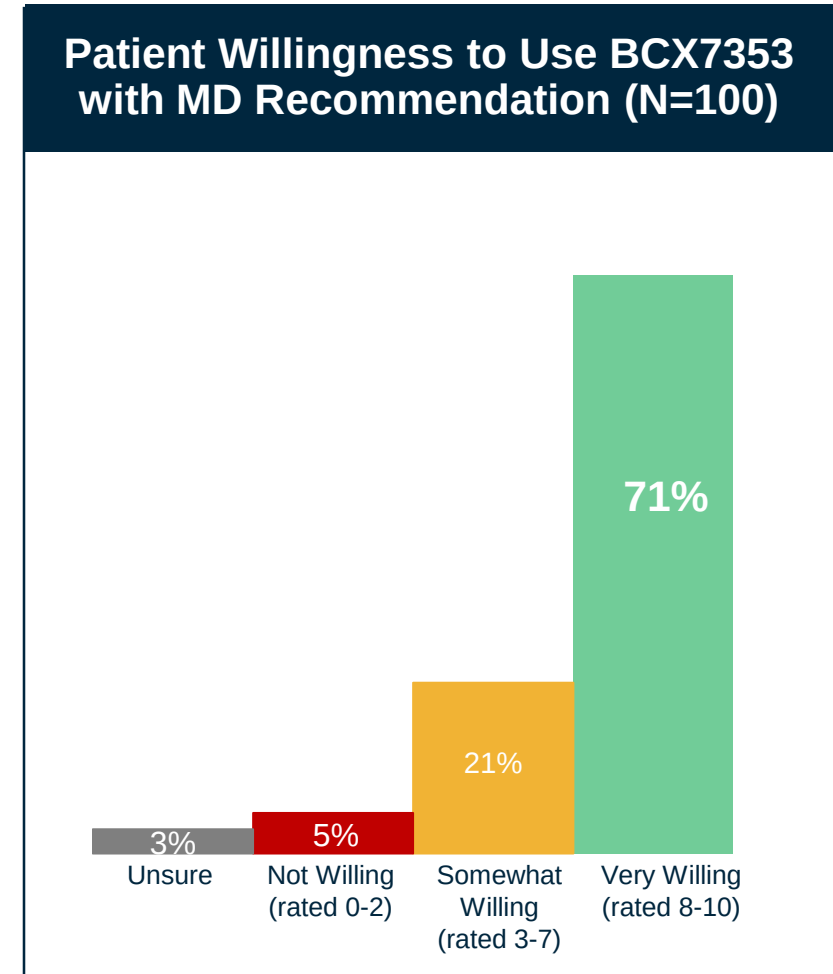
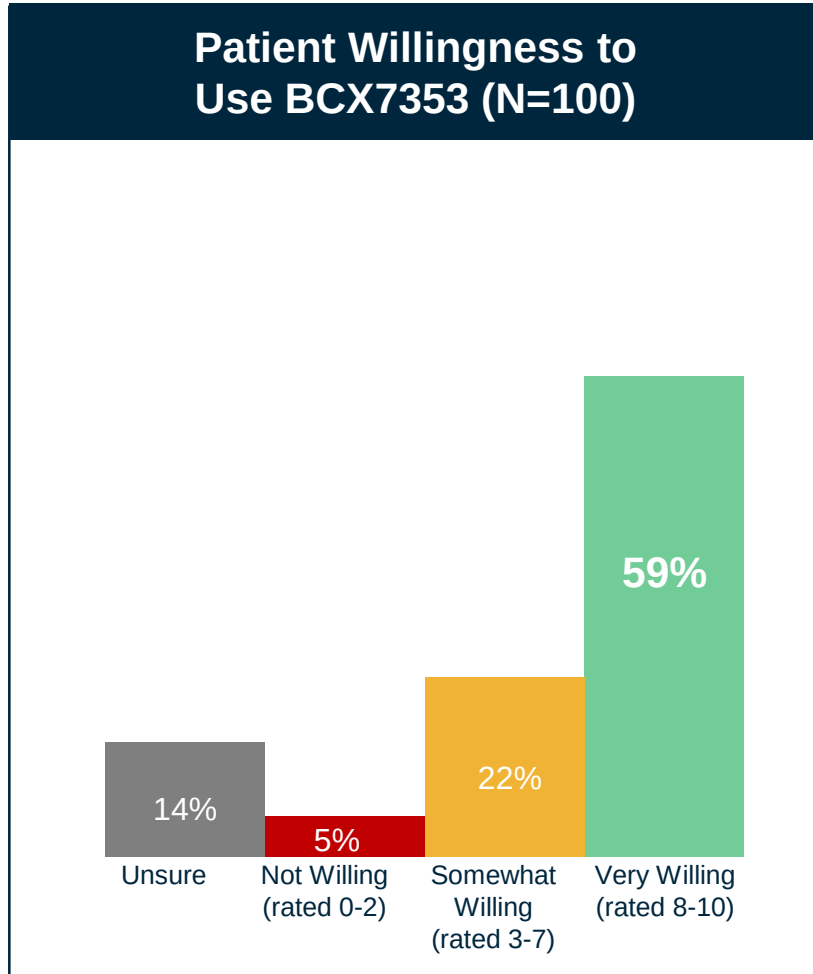
Respondents Viewed a Blinded Profile of BCX7353 Based on 24-week Results from APeX-2

Indication	<u>Prophylactic</u> 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

Strong HAE Patient Demand for BCX7353:

59% of Patients Expressed High Willingness to use BCX7353

Rises to 71% with Physician Recommendation

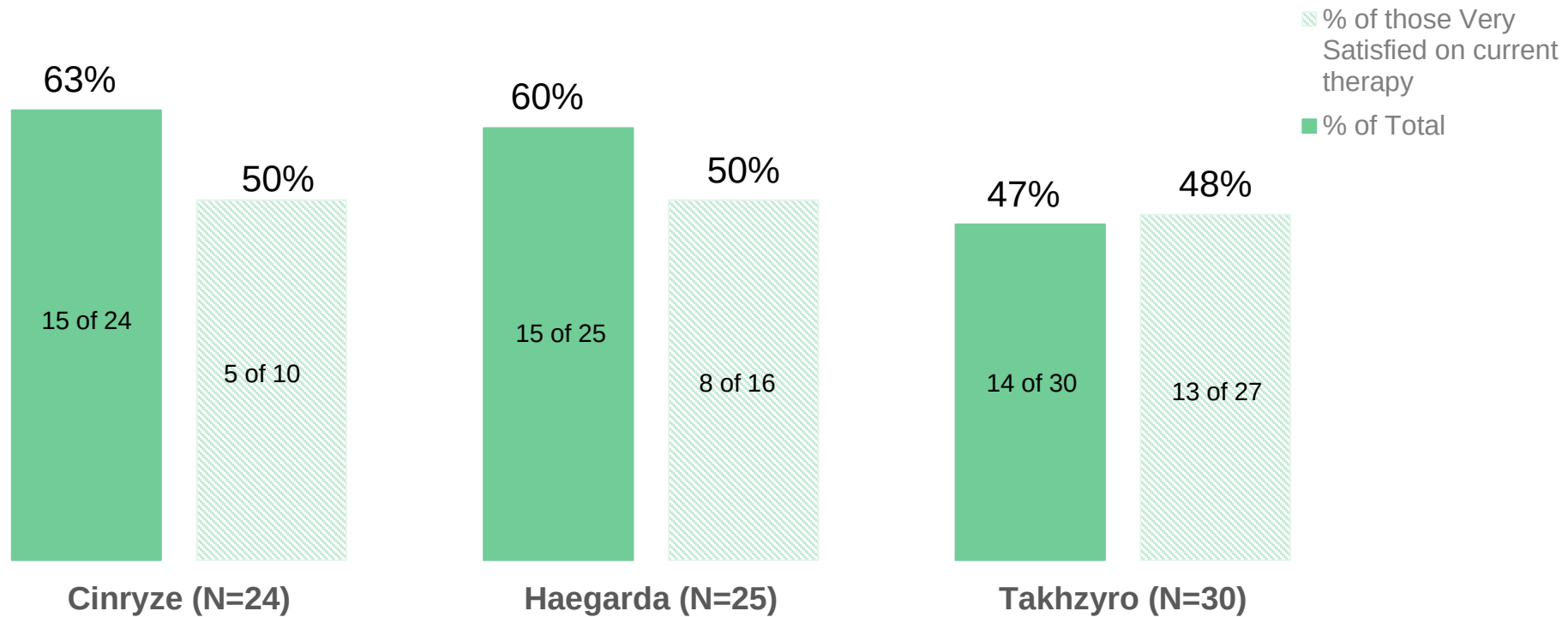


All Qualified HAE Patients (n=100)

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

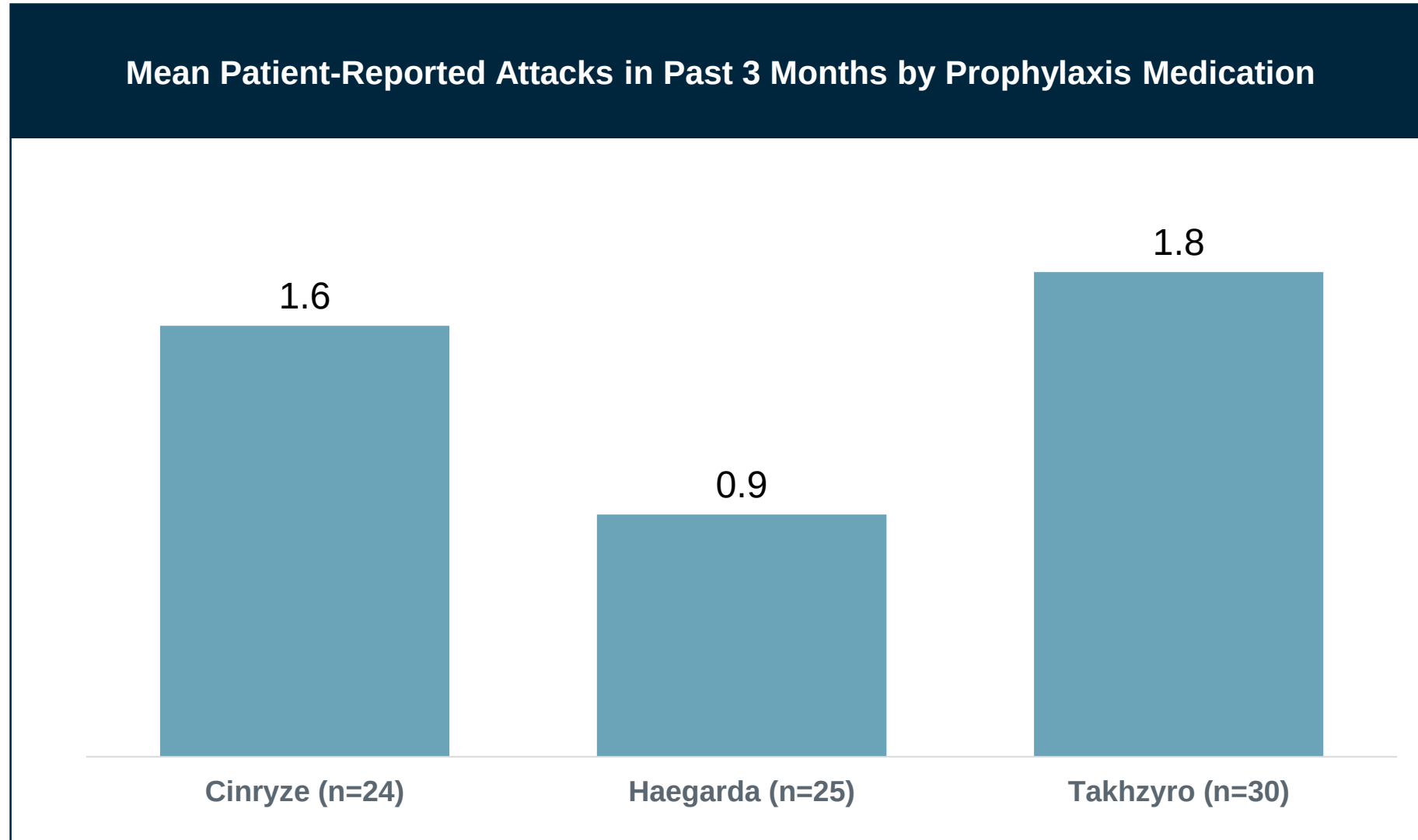
Prophylaxis Patients are Very Willing to Use BCX7353—Even Those Very Satisfied with their Current Injectable Prophylactic Treatment

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"

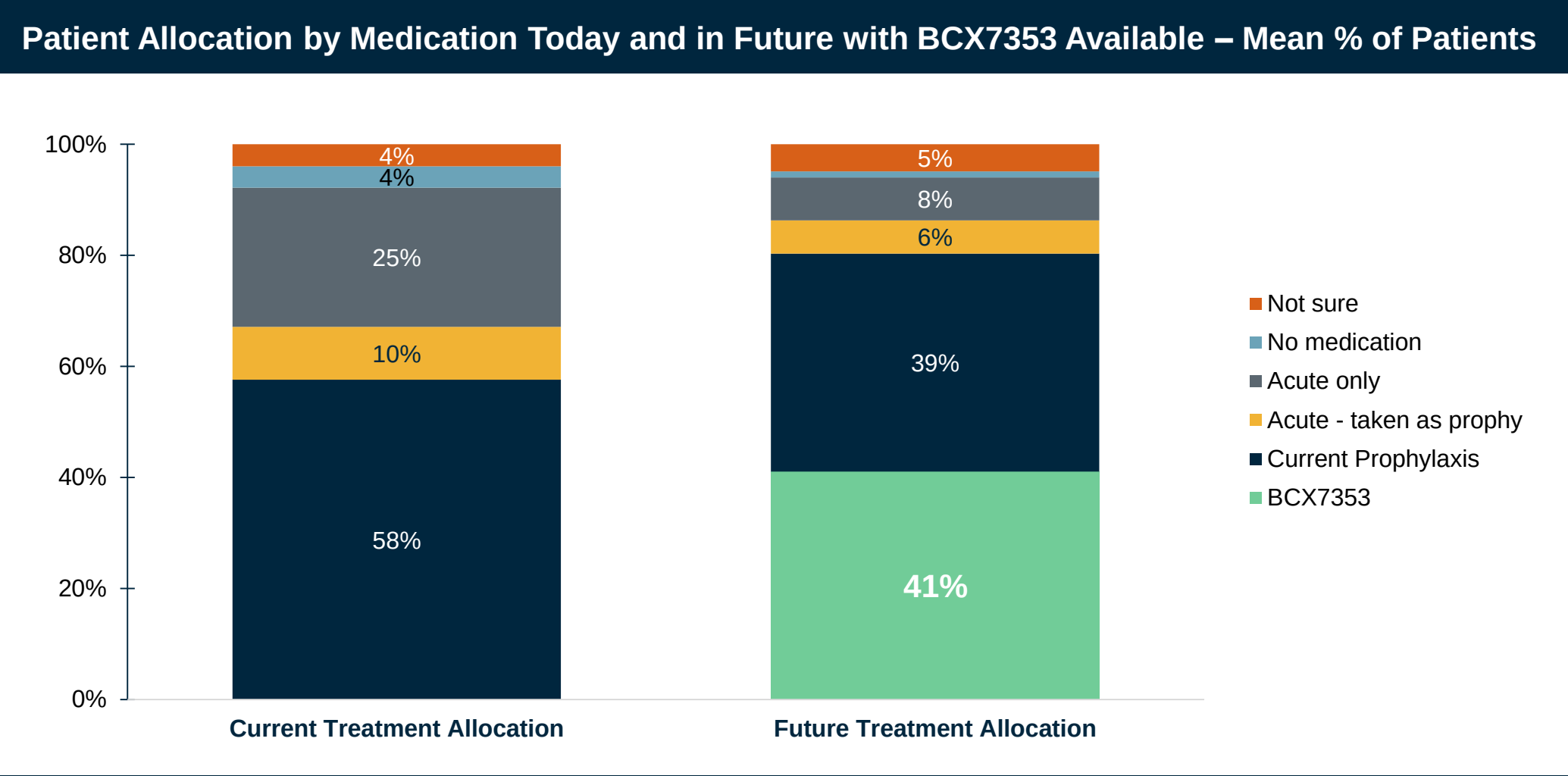
Patients Report Breakthrough Attacks with Injectable/Infused Treatments



Currently Taking Medication Prophylactically

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

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



All Qualified Respondents (n=175)

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

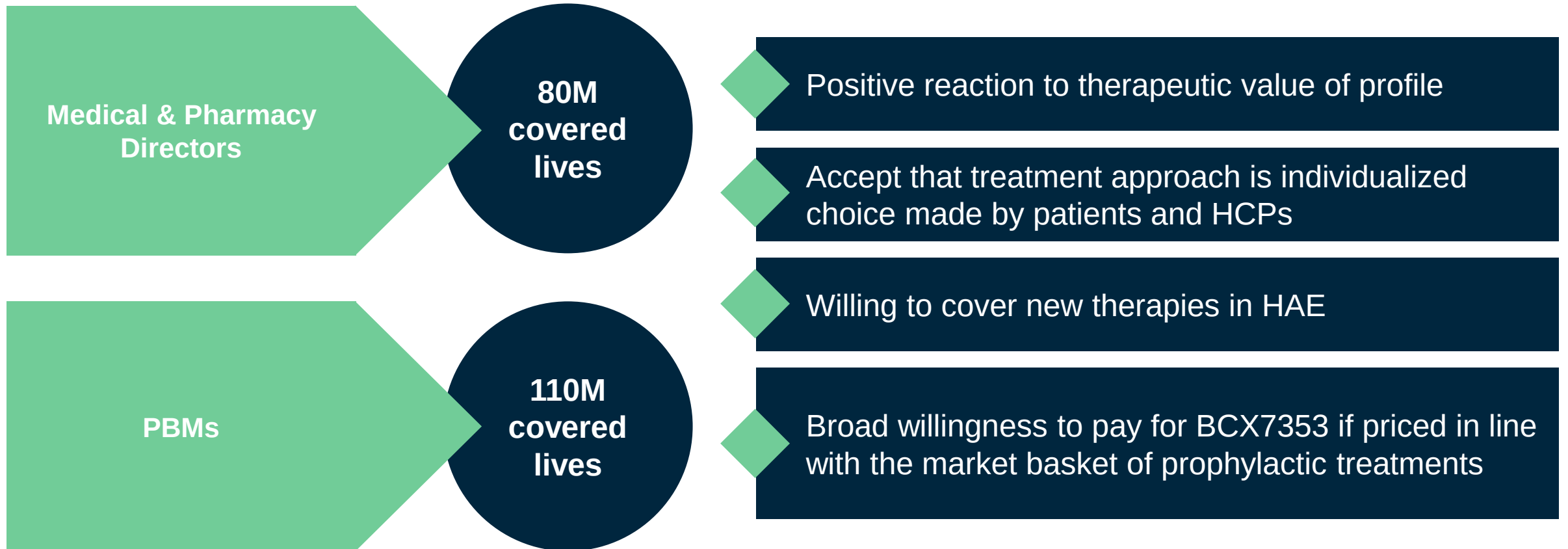


Physicians' expectations in market research	~50% of future use of berotralstat will come from patients switching from other prophylaxis treatments
APeX-2 enrollment	44% of patients treated previously with injected or infused C1 inhibitor prophylaxis
APeX-S enrollment in the United States	~50% of patients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

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

Efficacy	<i>"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."</i> <i>"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> <i>"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."</i>
Tolerability	<i>"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]."</i>
Less burden and improved quality of life	<i>"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> <i>"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."</i> <i>"After several years of being a pincushion it was nice to be able to take a pill"</i> <i>"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."</i> <i>"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."</i>

US Payors Anticipate Providing Coverage for Berotralstat



Berotrastat 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 Berotrastat 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		

Ready for Successful Commercial Launch



Sales force hired + trained

- Average 20 years of experience, including 8 in rare disease
- Top performers



Medical Affairs team deployed and engaging with the KOL community



Patient access/support systems in place



Robust dual-source supply chain to support commercial launch

Multiple Potential Global Approvals in 2020-2021



Orphan Drug 2017

*NDA accepted
PDUFA Date: 12/3/20*

2020



Sakigake 2015

*JNDA accepted
Approval Dec 2020*

2020



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

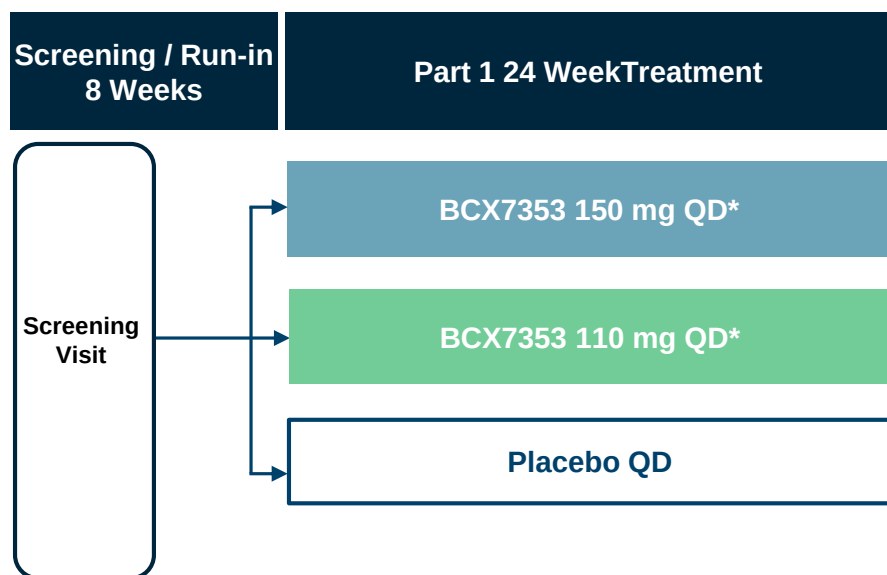
Orphan Drug 2018

*MAA accepted Mar 2020
CHMP Opinion: ~12 mo*

2021

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

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



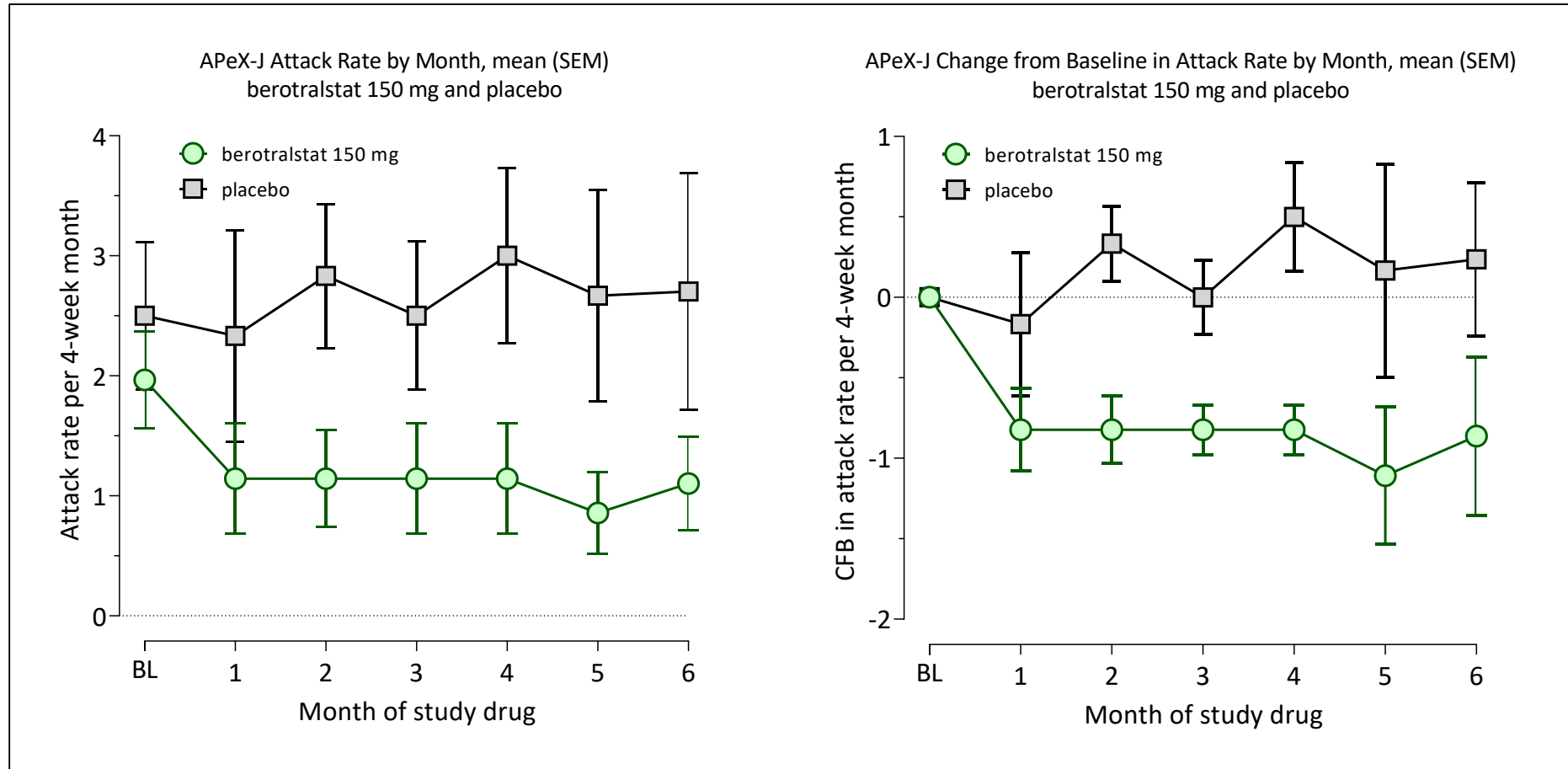
Primary analysis

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



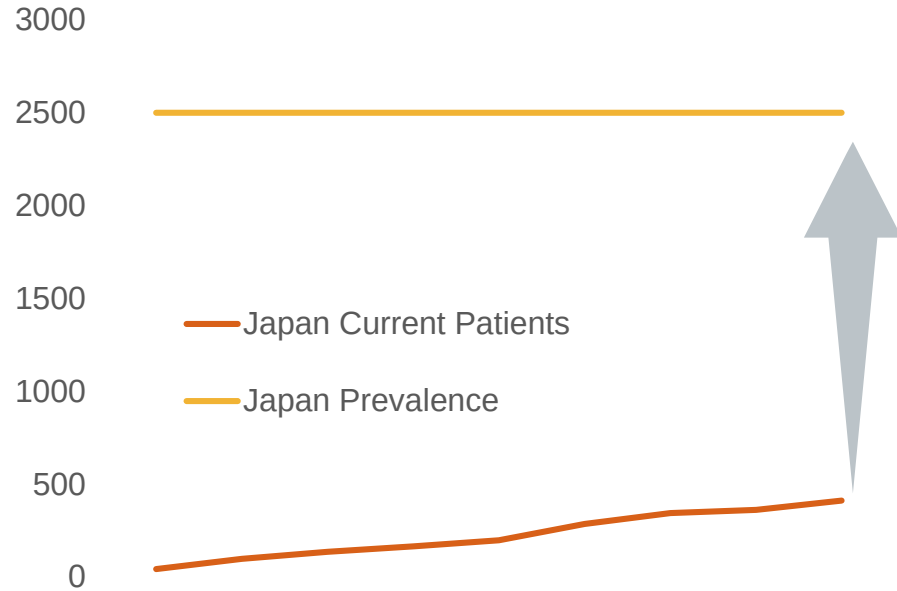
Overall Safety Summary: Berotralstat was Safe and Generally Well Tolerated

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



Japanese Market Growth Potential



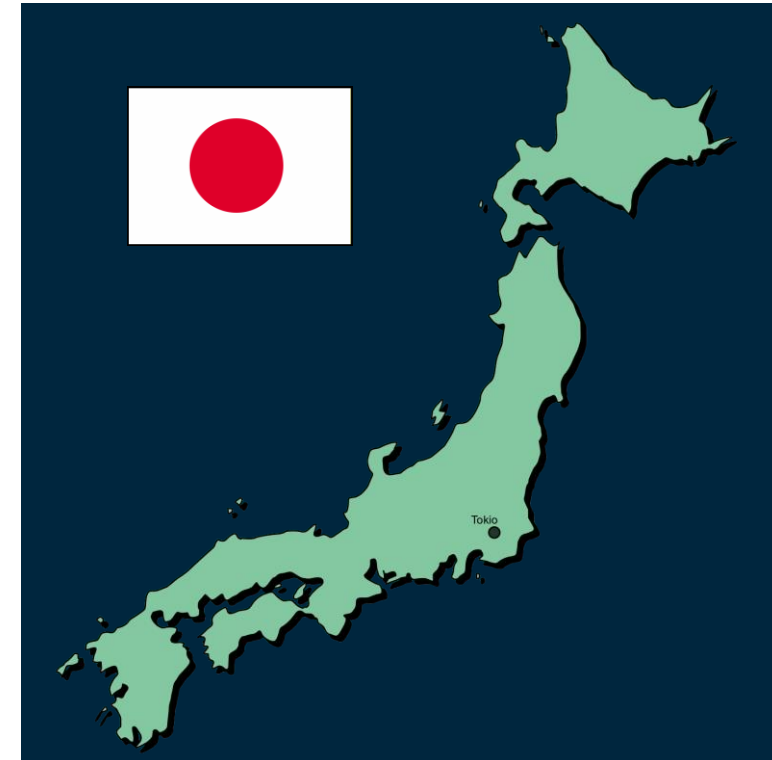
Source: HAE-J, Presentation: HAE Global Conference, 2016

- 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 with approval decision expected December 2020



Goal for BCX9930: Oral Factor D Inhibitor as Monotherapy for Multiple Complement-mediated Diseases

Factor D is an ideal target:

Required for the alternative pathway (AP) to work

Target is the same in PNH, nephritis, and other AP diseases

Circulating Factor D levels are the lowest of any complement pathway enzyme

Levels do not increase with inflammatory illnesses

Unique enzyme structure enables design of inhibitors with better specificity against other serine proteases

Application to BCX9930 Development:

Doses of BCX9930 that block Factor D will inhibit the AP independent of the disease setting

Proof of concept in PNH provides POC for other diseases of the alternative pathway

Less drug required for inhibition compared to other complement targets

No dose adjustment when patients get illnesses like influenza

Can lead to a better safety margin

Significant Unmet Need in PNH Due to Uncontrolled Hemolysis

Anemia and PNH Symptoms Persist in Many Patients Despite C5 Inhibitor Treatment

C5 Inhibitor	Hb not Stabilized	Fatigue	Hemoglobinuria	Dyspnea
Ravulizumab	32%	29%	10%	14%
Eculizumab	35%	30%	9%	14%

J. W. Lee *et al.*, *Blood* **133**, 530-539 (2019)

Extravascular Hemolysis Develops in Most PNH Patients with C5 Inhibitor Treatment

- *All patients treated with eculizumab had opsonized RBCs* *C3+ RBC: 41/41 (100%)*
- *Most patients had persistent hemolysis*

A. M. Risitano *et al.*, *Blood* **113**, 4094-4100 (2009)

- ***Effective Alternative Pathway complement inhibition with BCX9930 will control both intravascular and extravascular hemolysis regardless of bone marrow function****

*56% of >4,000 patients in the PNH registry had aplastic anemia with PNH, with impaired bone marrow function

H. Schrezenmeier *et al.*, *Ann Hematol* **99**, 1505-1514 (2020)

Factor D Inhibition in PNH Offers the Advantage of Controlling Both Intravascular (IVH) and Extravascular (EVH) Hemolysis

Goal of Total Hemolytic Control

IVH Control

↓ LDH



EVH Control

↓ Opsonized RBCs

Patient Outcomes from Controlled Hemolysis over Time

Relieve Anemia

↑ Hemoglobin

Reduce PNH Clinical Symptoms

↓ Appearance of symptoms

Avoid Transfusions

↓ Transfusions

Goal of Ongoing Dose-ranging Study: Choose Optimized Dose for Advanced Development as Monotherapy

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 2: n = up to 4

200 mg BID days 1-14

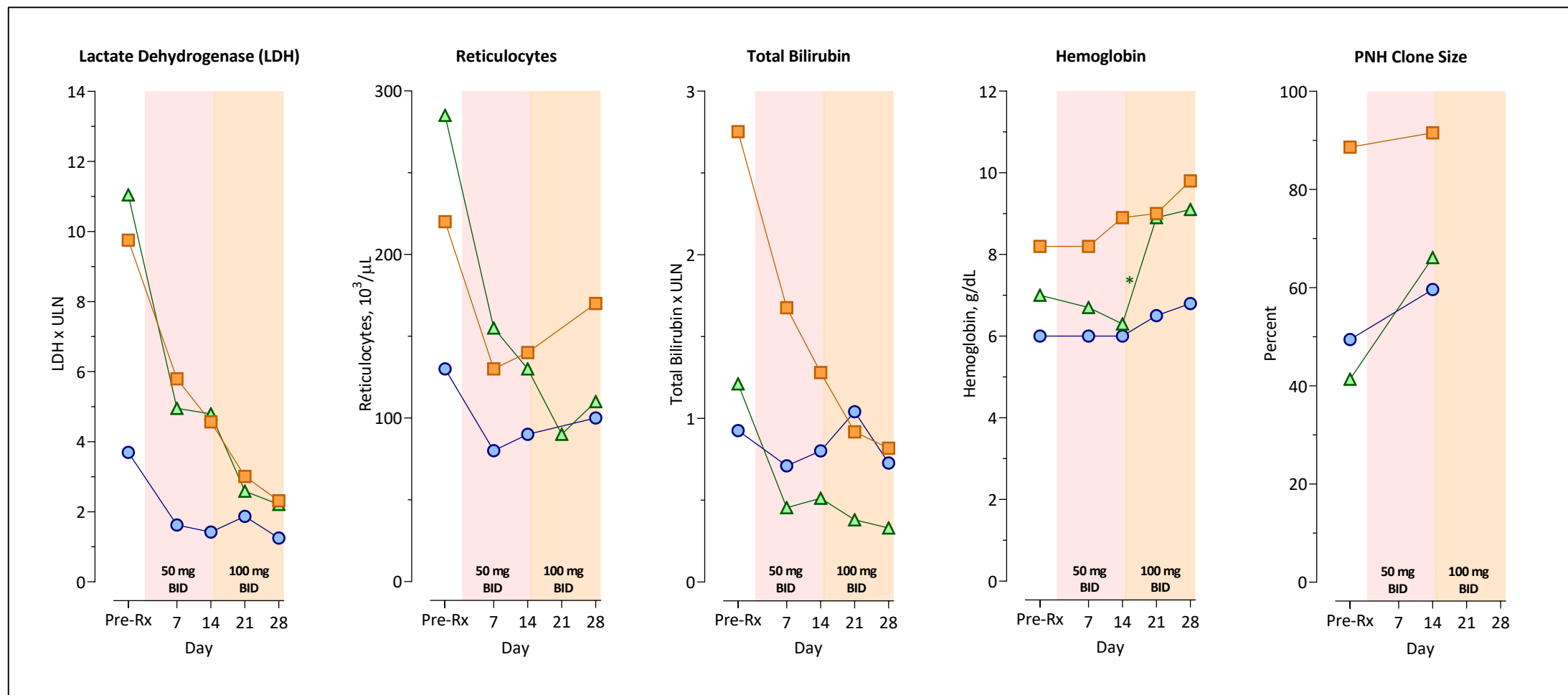
400 mg BID days 15-28

*Subjects
benefiting
from study
drug may
continue on
treatment*

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
$LDH \times ULN$	9.8	11.0	3.7
Hemoglobin, g/dL	8.2	7.0	6.0
Reticulocytes, 10^3 cells/ μ L	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
<i>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.</i>			

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

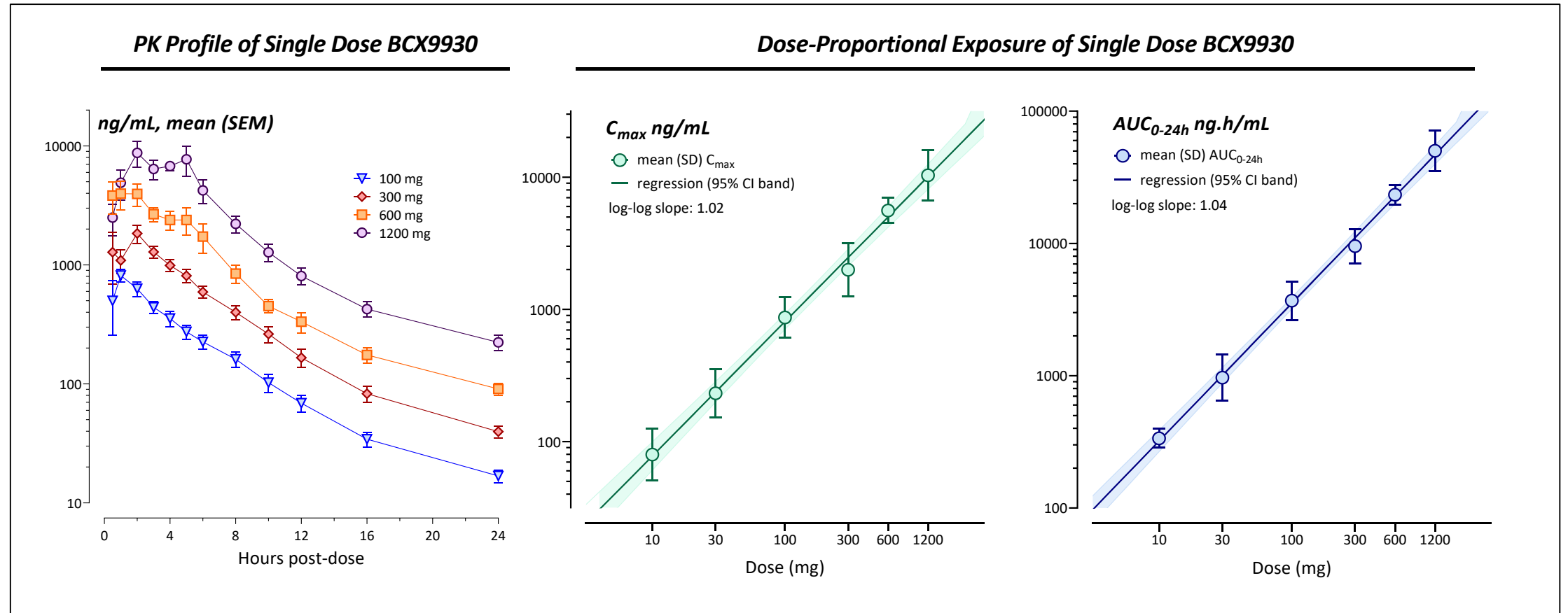
Activity in PNH at low doses, n=3

- 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

- 200/400 mg bid cohort for C5-inhibitor naïve patients, data update expected Q3 2020
- C5-inhibitor poor responders in 200/400 mg bid cohort, data update expected by YE 2020

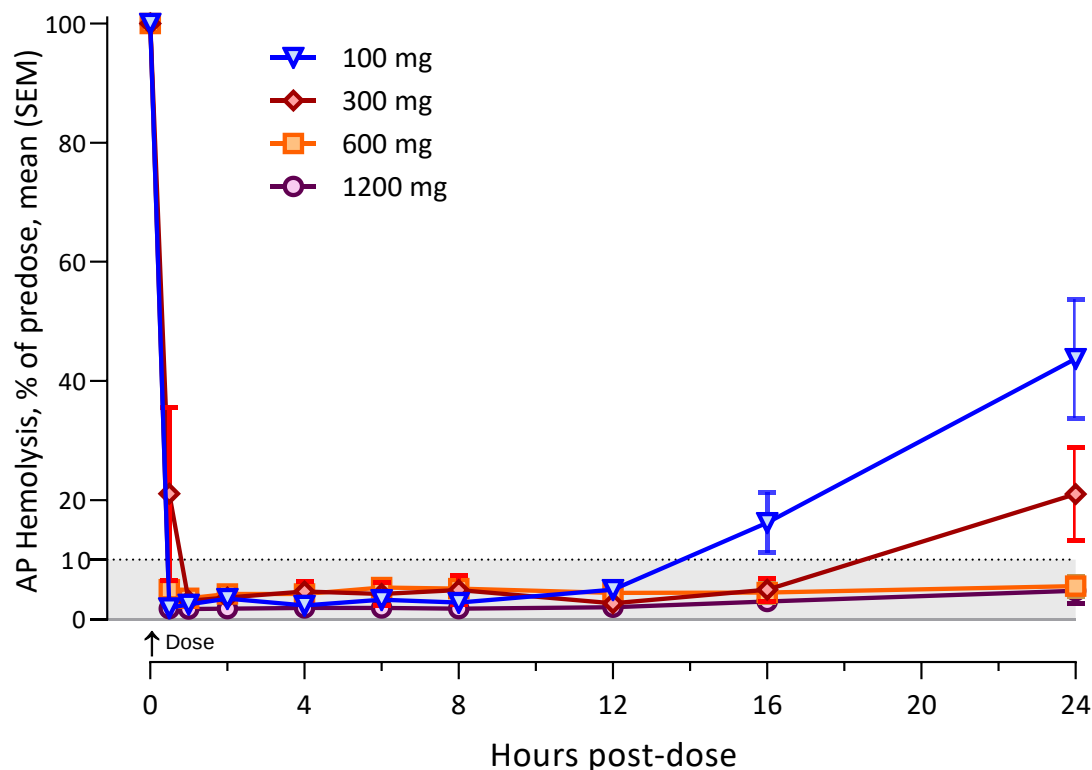
Single Dose PK Profile of Oral BCX9930 in Healthy Subjects



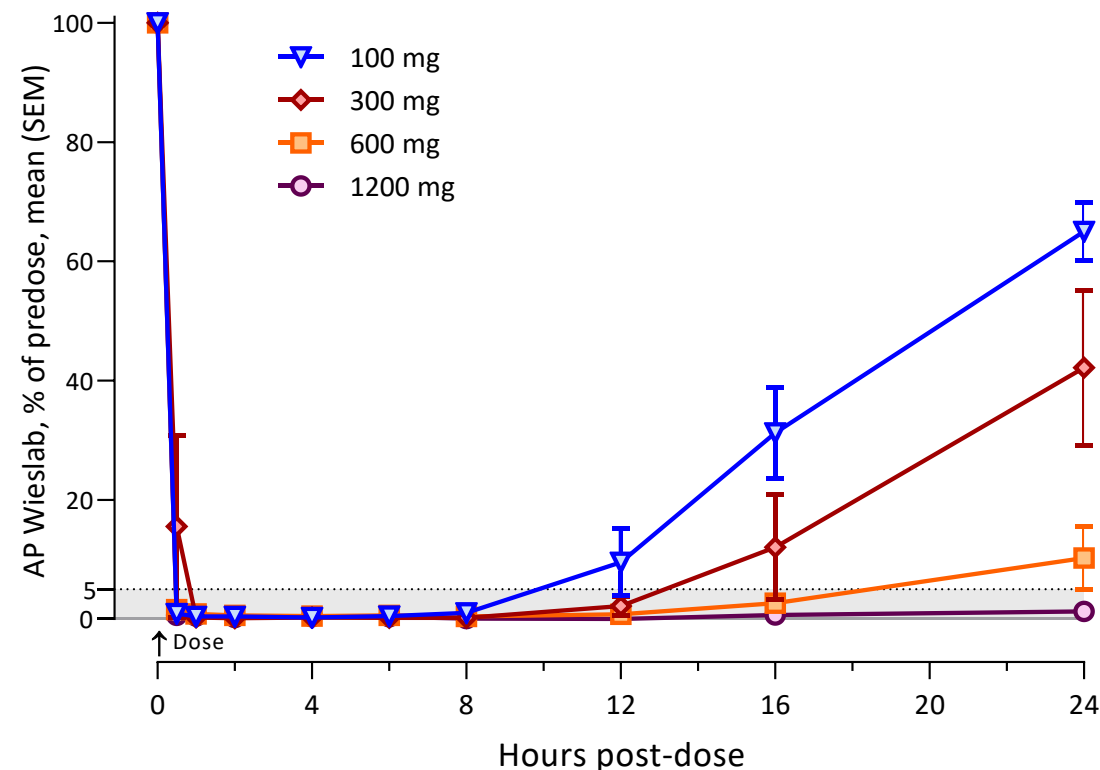
Suppression of AP Activity After Single Oral Doses of BCX9930

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

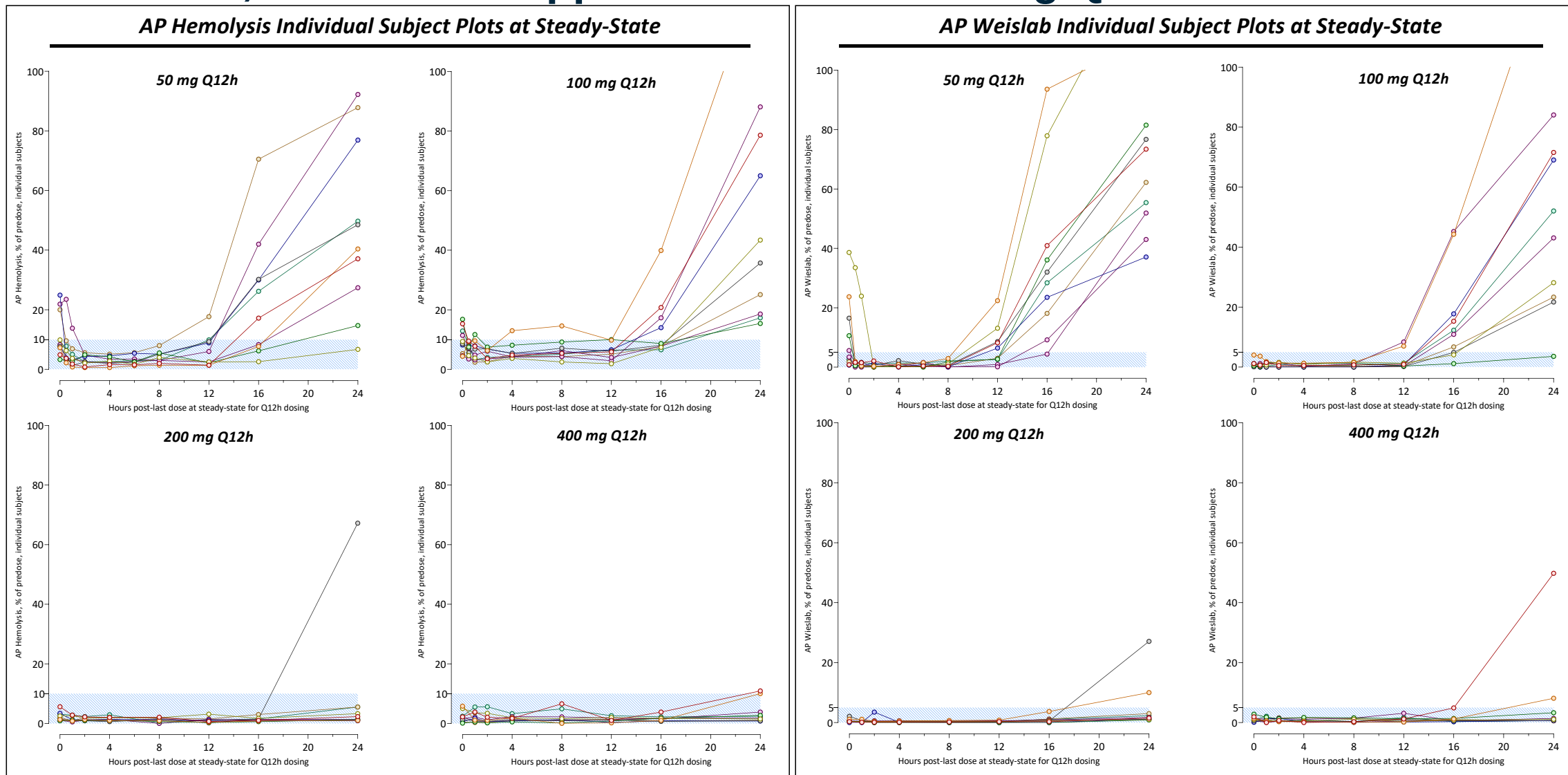
Assay: AP Hemolysis



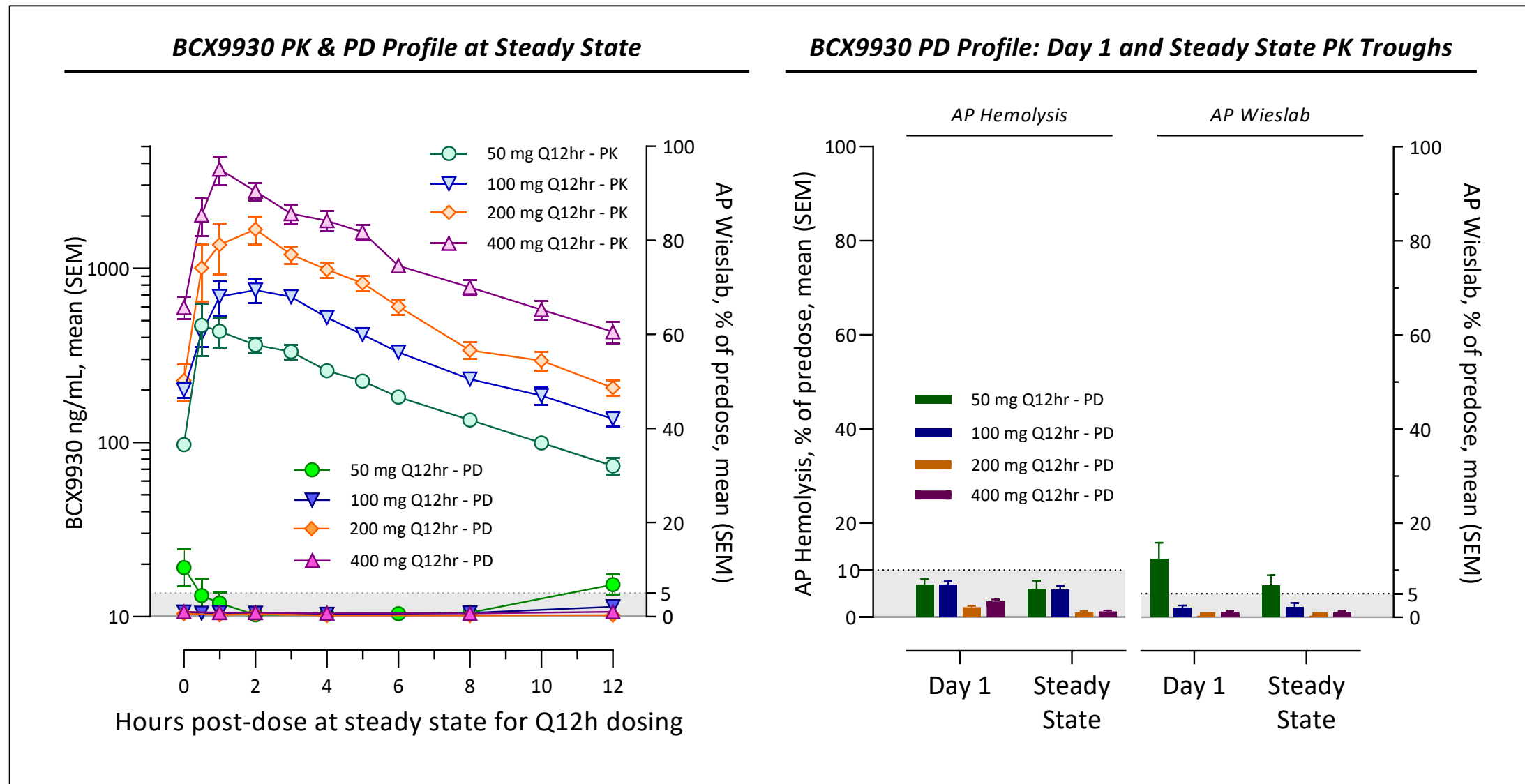
Assay: AP Wieslab



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



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



COVID-19 Antiviral Status Update: Galidesivir (BCX4430)

New \$47M of NIAID Contract Awards to Support:

- Completion of Parts 1 & 2 of ongoing clinical trial of galidesivir in Brazil
- Conducting a phase 2 trial of galidesivir in non-hospitalized COVID-19 patients at high risk for developing severe disease and complications
- Conducting a clinical pharmacology trial of galidesivir to determine appropriate dosing in patients with renal impairment
- Increasing the supply of galidesivir

- Part 1 of trial in Brazil has advanced to final cohort
- Data from Part 1 + decision on dose for Part 2 expected in Q4 2020

- Galidesivir has shown activity against >20 RNA viruses in 9 different families, including coronaviruses¹
- Animal and *in vitro* studies against SARS-CoV-2 ongoing

\$ 129 M of program support to date:



National Institute
of Allergy and
Infectious Diseases



Cash position & 2020 guidance (in millions)

Cash & investments at December 31, 2019	\$138
Cash & investments at March 31, 2020	\$115
Cash & investments at June 30, 2020	\$192
Senior credit facility	\$50
REVISED FY 2020 GUIDANCE	
Net operating cash utilization	\$150-165
Operating expenses ^A	\$180-195

A - Excludes equity-based compensation.

September 2020 Corporate Presentation

