Jefferies London Healthcare Conference

Jon Stonehouse

Chief Executive Officer

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Forward-Looking Statements

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Delivering extraordinary
Empowering ordinary

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

Robust Pipeline Through In-house Innovation

	Lead Optimization	Pre-clinical	Phase 1	Phase 2	Phase 3	Filed	Approved
STRATEGY: Develop oral therapies for life-threatening, rare diseases							
BCX7353 – Oral Capsule (Prophylactic HAE)							
BCX7353 – Oral Formulation (Acute HAE)							
BCX9930 – Oral Factor D Inhibitor (Complement-Mediated Diseases)							
BCX9250 (FOP)							
SUPPORTING ASSETS: Externally funded, potential for capital infusions							
RAPIVAB® (peramivir injection)*							
Galidesivir (Broad spectrum antiviral) I.V.							

*Licensed to Seqirus, Shionogi and Green Cross

BCX7353:
Oral, Once Daily to Prevent HAE Attacks

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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

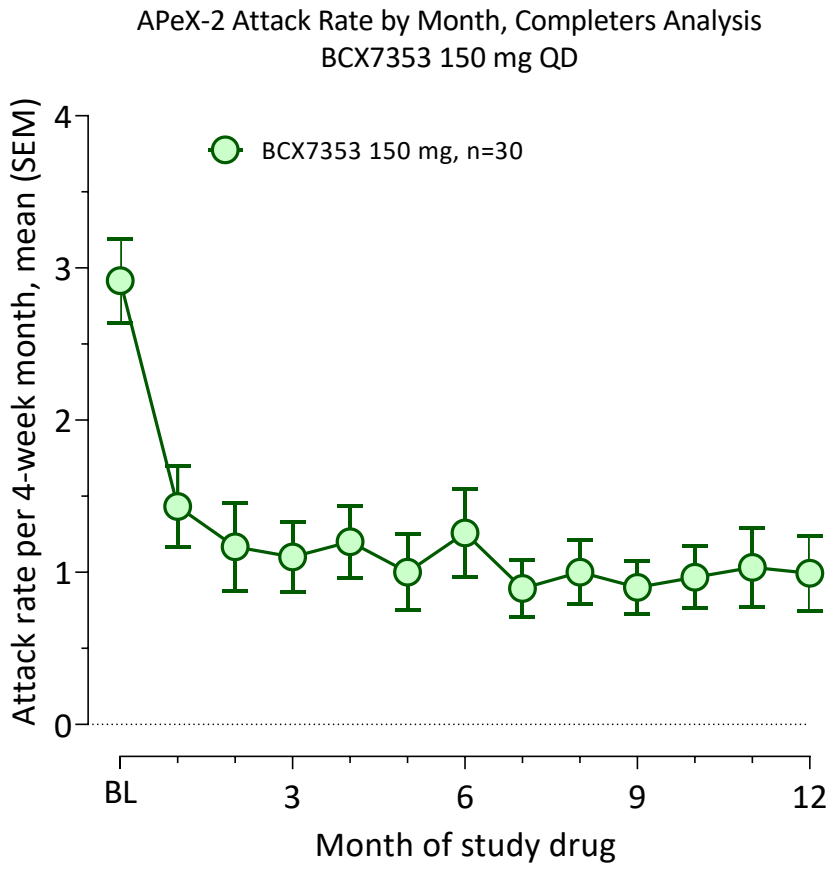
- *Daily oral BCX7353*
- *Regular clinic visits*
- *Safety assessments*
- *HAE attacks recorded*

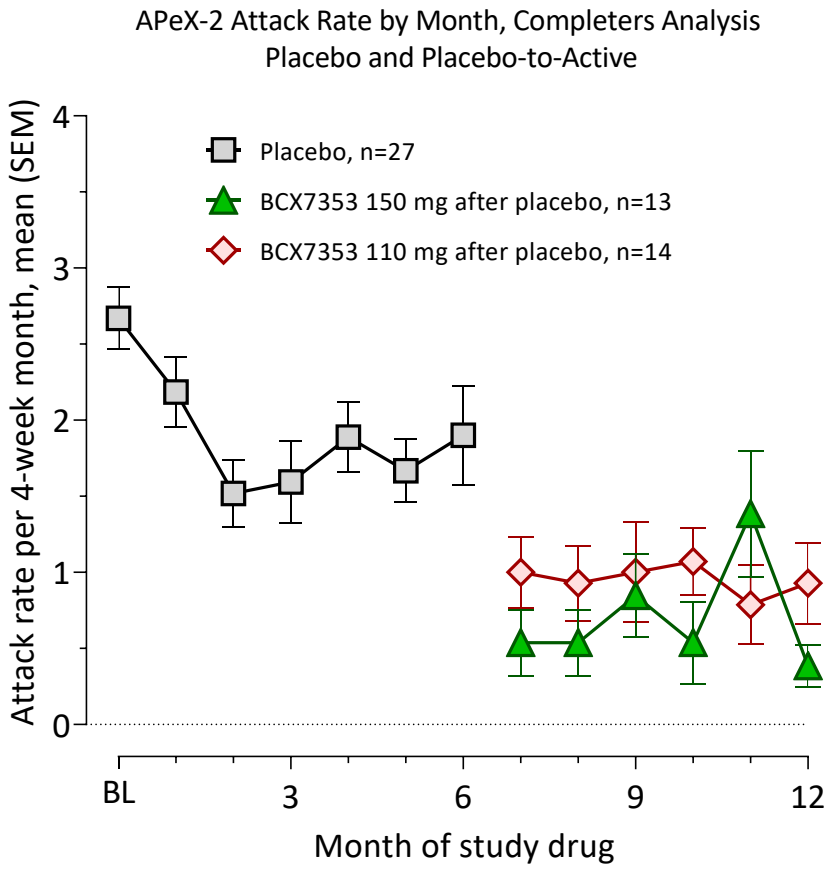
Follow-up

48-week analysis

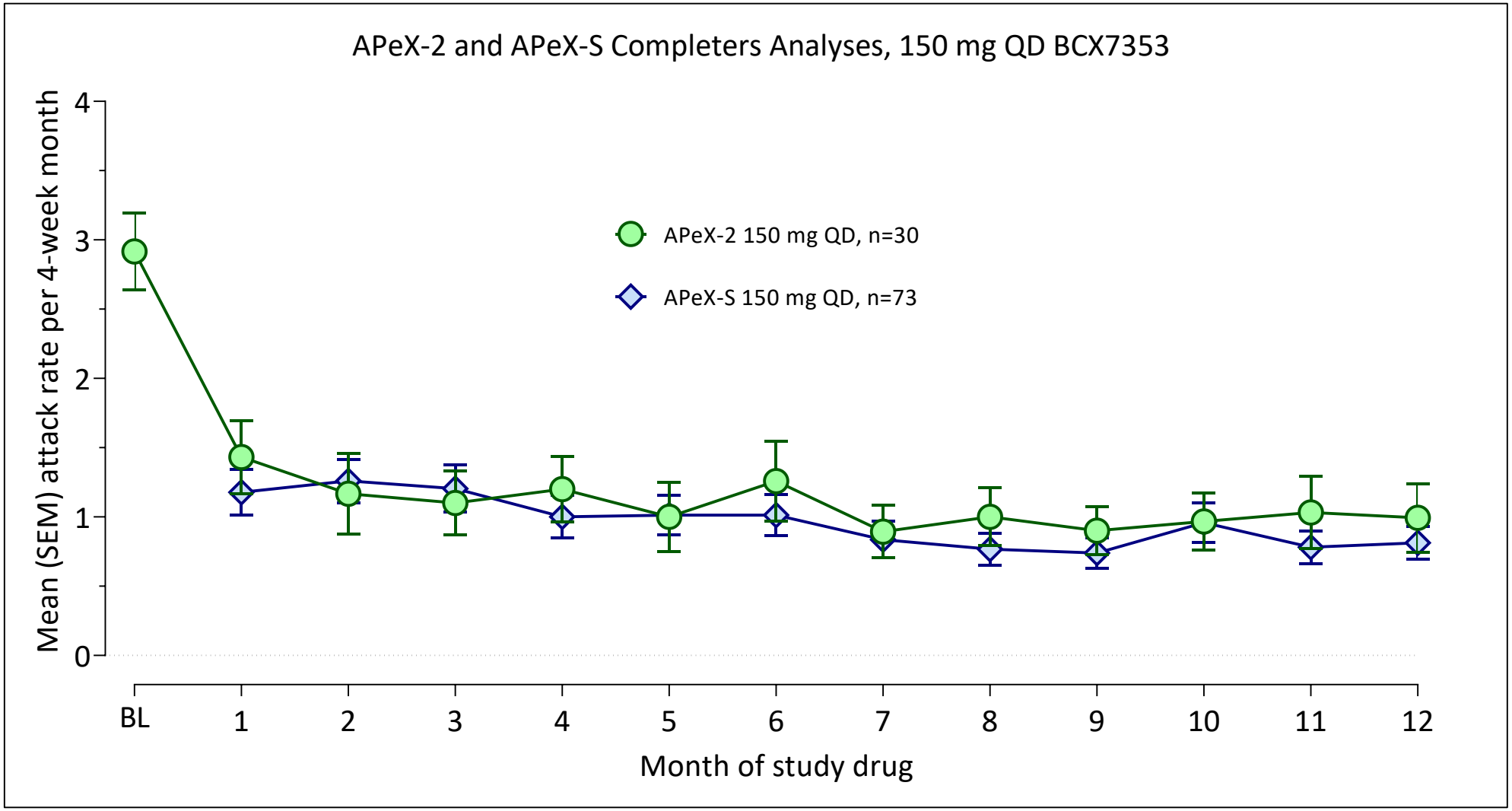
Subjects Enrolled and Follow-up	**BCX7353 110 mg**	**BCX7353 150 mg**
Subjects enrolled [Safety Population]	100	127
Subjects continuing on study, not yet reaching 48 weeks of study drug	44	23
Subjects who discontinued study drug before 48 weeks	26	31
Subjects who completed 48 weeks of BCX7353 [Completers 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

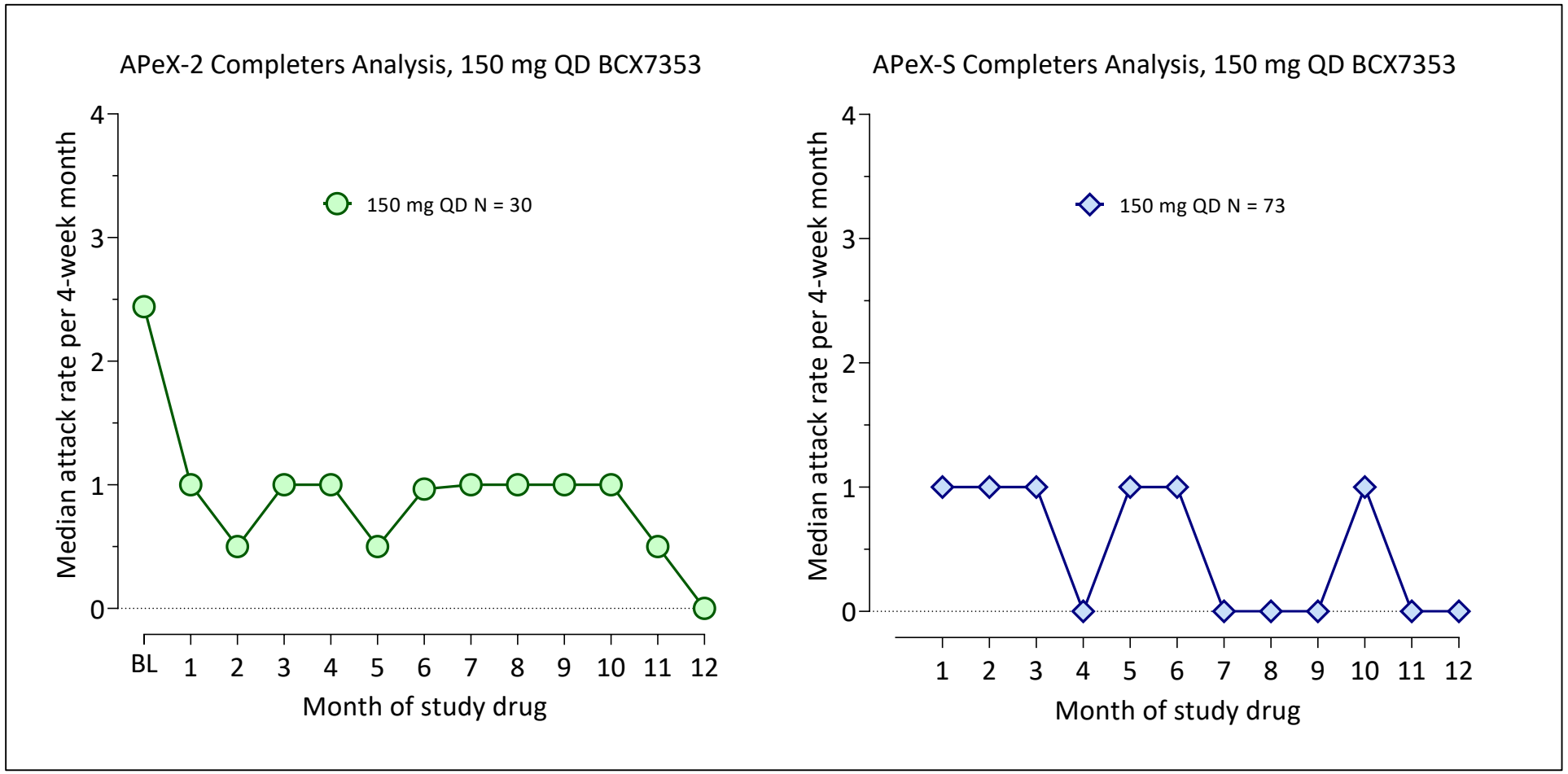




Consistent Mean Attack Rates in APeX-2 and APeX-S



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

Data Supports 4-week Washout Period for Current Androgen Users

APeX-S Analysis	BCX7353 110 mg				BCX7353 150 mg			
Prior Androgen Rx Status	*All Subjects*	*Discontinued ≤ 1 month before BCX7353*	*Discontinued > 1 month before BCX7353*	*Never used*	*All Subjects*	*Discontinued ≤ 1 month before BCX7353*	*Discontinued > 1 month before BCX7353*	*Never used*
Number of Subjects	*N = 100*	*N = 26*	*N = 43*	*N = 31*	*N = 127*	*N = 23*	*N = 61*	*N = 43*
Subject Incidence of Post-Baseline ALT > 3xULN	*9 (9.0%)*	8 (30.8%)	1 (2.3%)	0	*6 (4.7%)*	6 (26.1%)	0	0

Assessment:

- *There is a strong association of recent discontinuation of androgens with post-baseline elevations in ALT*

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

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

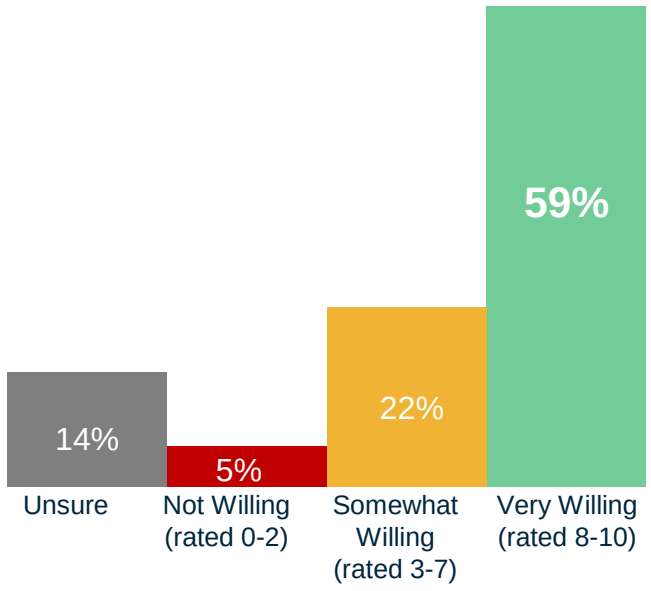
Indication	Prophylactic treatment of HAE for patients 12 years and above
Dosage	Take 1 capsule by mouth once per day
Clinical trial design	Patients who were experiencing an average of 3 HAE attacks per month took Treatment X or a placebo (an inactive drug often used in clinical trials) for 6 months
Efficacy	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.

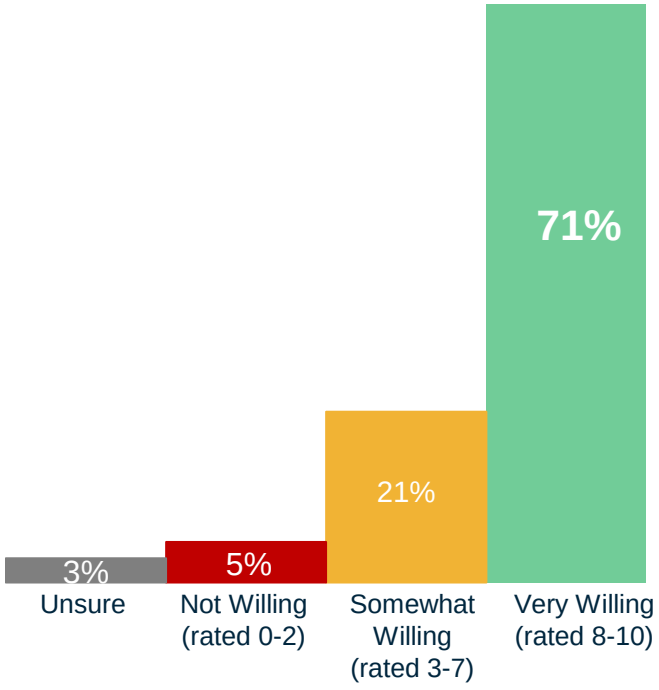
Strong HAE Patient Demand for BCX7353:

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

Patient Willingness to Use BCX7353 (N=100)



Patient Willingness to Use BCX7353 with MD Recommendation (N=100)

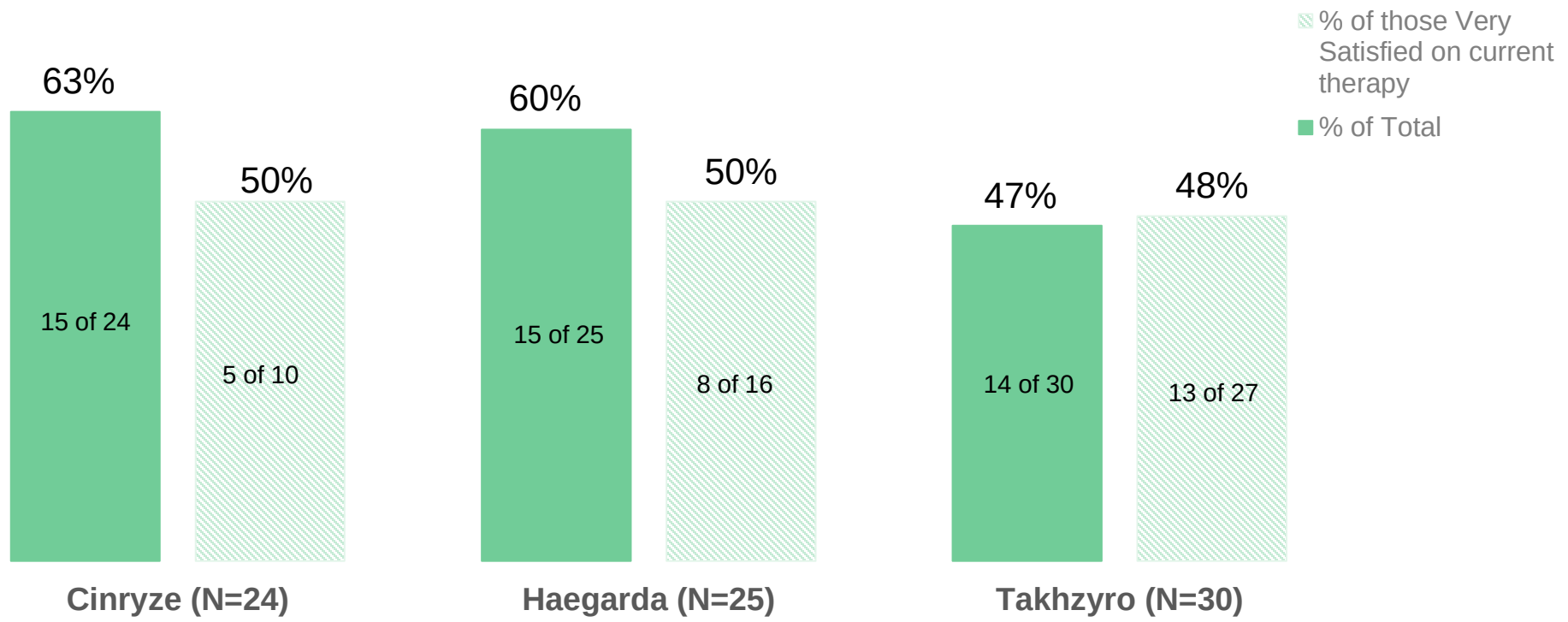


All Qualified HAE Patients (n=100)
Rated on a scale where a "0" indicates "Not at all willing", and a "10" indicates "Extremely willing"

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

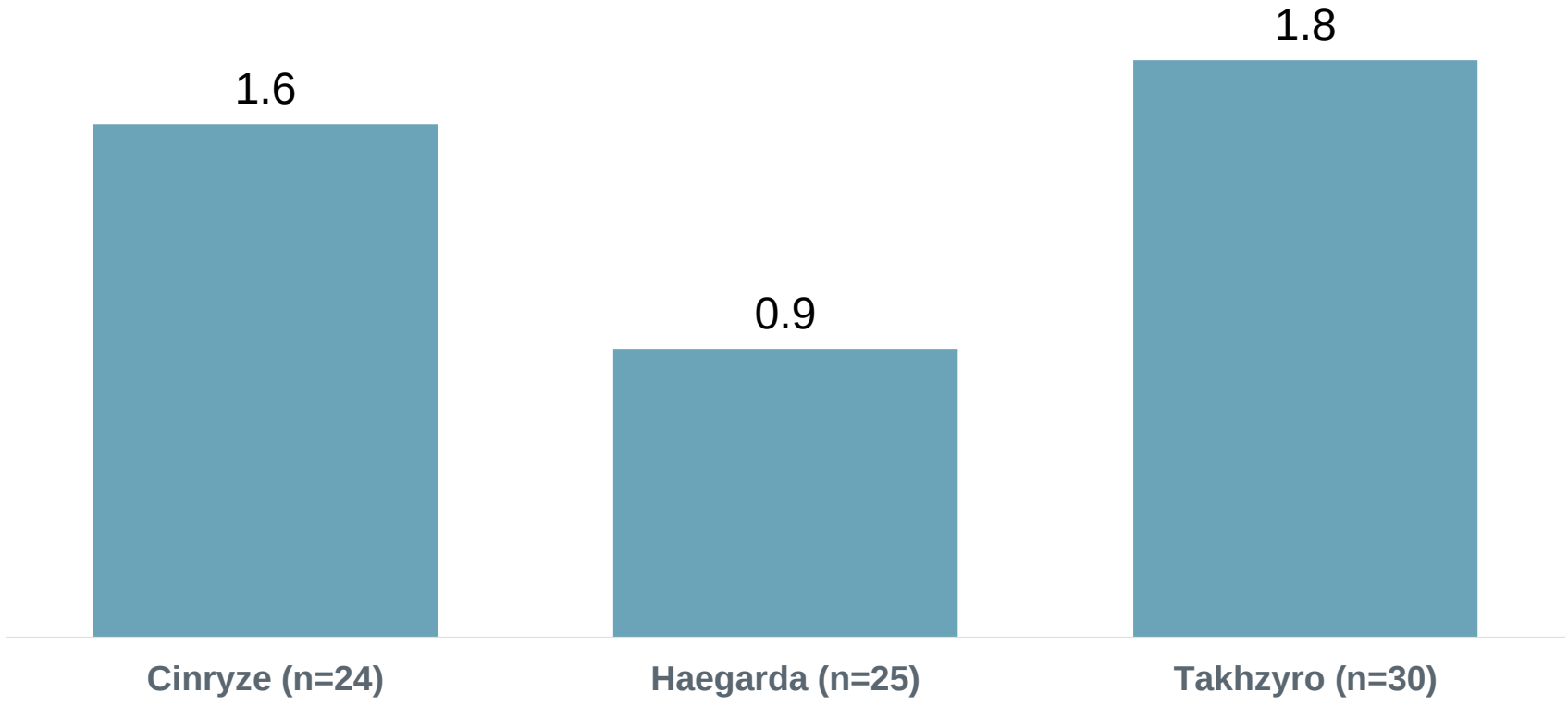


All Current Prophylaxis Users- "Very Willing" & "Very Satisfied" = Top 3 Box (rated 8,9,10 on 10 point scale)
Willingness rated on a scale where a "0" indicates "Not at all willing", and a "10" indicates "Extremely willing"
Satisfaction with current treatment rated on a scale where a "0" indicates "Not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

Mean Patient-Reported Attacks in Past 3 Months by Prophylaxis Medication



Currently Taking Medication Prophylactically

Source: BioCryst Proprietary Market Research Study, 2019.

Patients Are Coping With Their Injectable Therapies

They Want the Ease that Only BCX7353 can Offer

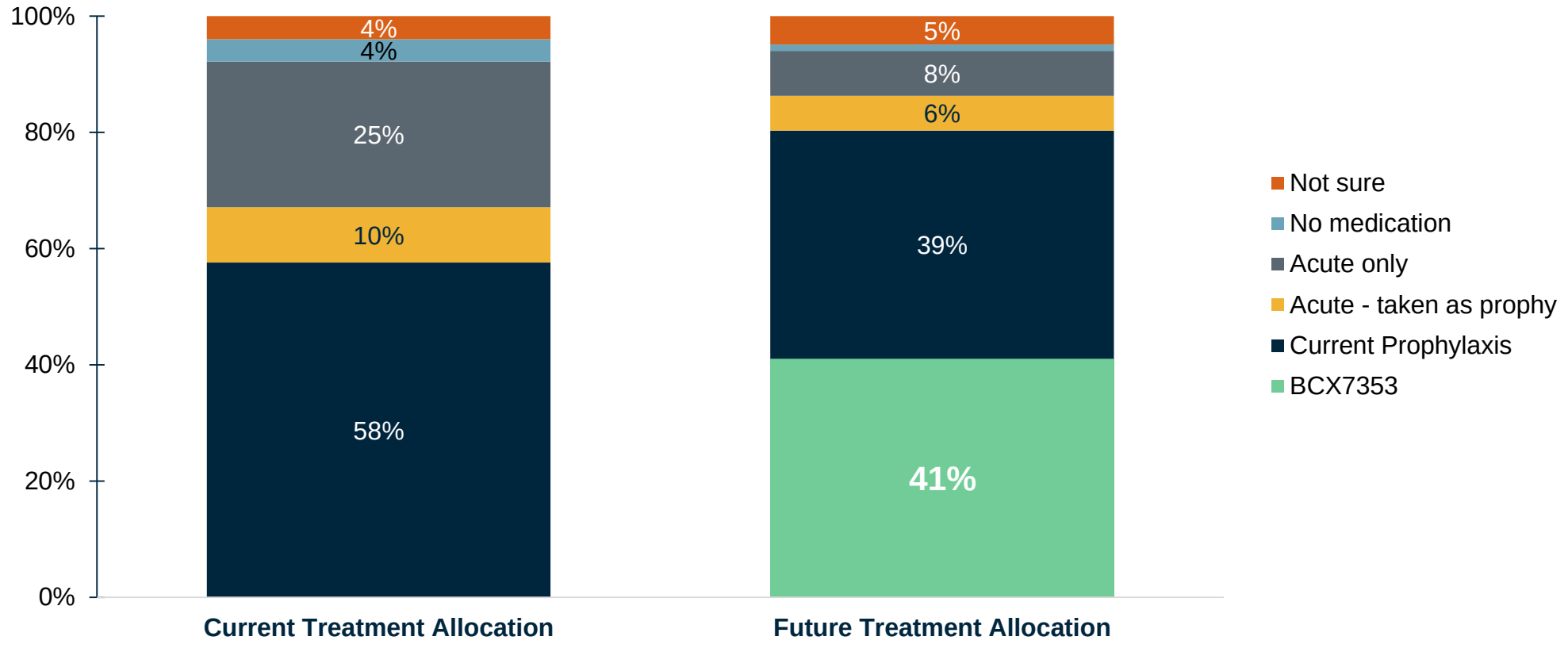
Time saving	*"Make HAE less a part of my life...less time consuming...getting on with my day. A lot of times I am so rushed and **I don't have the time to do simple things**, like do laundry or go grocery shopping. Being able to do this quickly...this is one thing that will be a lot simpler and help 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 BCX7353

Medical & Pharmacy Directors → **80M covered lives**

PBMs → **110M covered lives**

- Positive reaction to therapeutic value of profile
- Accept that treatment approach is individualized choice made by patients and HCPs
- Willing to cover new therapies in HAE
- Broad willingness to pay for BCX7353 if priced in line with the market basket of prophylactic treatments

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

BCX7353 for HAE Prophylaxis: Data Supports Global Peak Market Opportunity >$500M

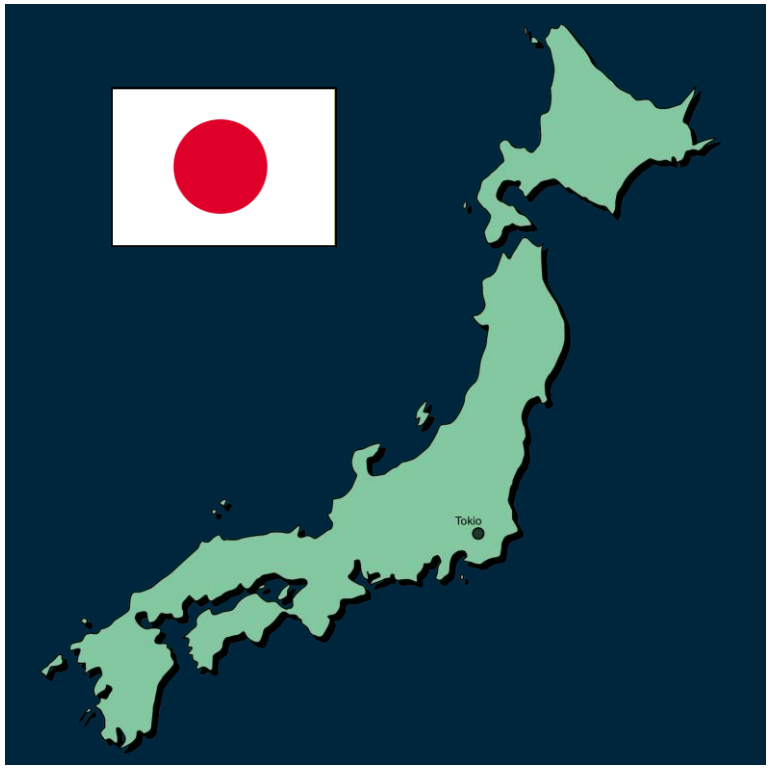
Clinical Data	Prevalence	Treatment Paradigm
Consistent, clinically meaningful benefit demonstrated through 48 weeks Safe and generally well-tolerated	~10,000 (US) HAE Patients ~7,500 diagnosed and treated	Physicians expect shift to ~80% prophylaxis

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

BCX7353 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
- No approved prophylaxis HAE treatments in Japan



Regulatory Agency Status for BCX7353

- Orphan Drug Designation 2017
- EOP2 2017
- NDA submission expected Q4 2019

- Orphan Drug Designation 2018
- National Scientific Advice 2018
- Scientific Advice Process (EOP2 Equivalent) 2017
- MAA submission expected Q1 2020

- UK Promising Innovative Medicine (PIM) 2018

- Formal Consultation Process (EOP2 equivalent) 2018
- Sakigake Designation 2015
- JNDA submission expected Q1 2020

BCX9930
Oral Factor D Inhibitor for Complement-mediated Diseases

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Over $10 Billion Global Market Opportunity

Significant pipeline potential for a differentiated oral complement inhibitor

*The only marketed complement inhibitor in 2018 is **IV-infused****

1

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

$3.6 billion

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

7+

*** SOLIRIS® (eculizumab) 2018 sales in paroxysmal nocturnal hemoglobinuria (PNH), atypical hemolytic uremic syndrome (aHUS), and generalized myasthenia gravis (gMG) reported 2/4/19**
**** Additional potential orphan indications for complement inhibitors include, but are not limited to, neuromyelitis optica (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

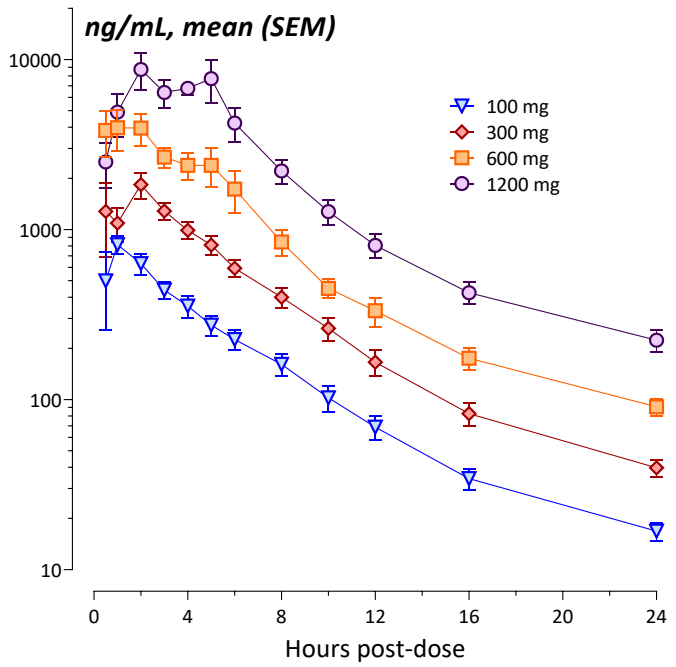
Part 1 – Single ascending dose
- Healthy subjects
- PK & PD
- Safety and tolerability
- 8 subjects per cohort
  6:2 active : placebo
- 6 dose levels
- Completed

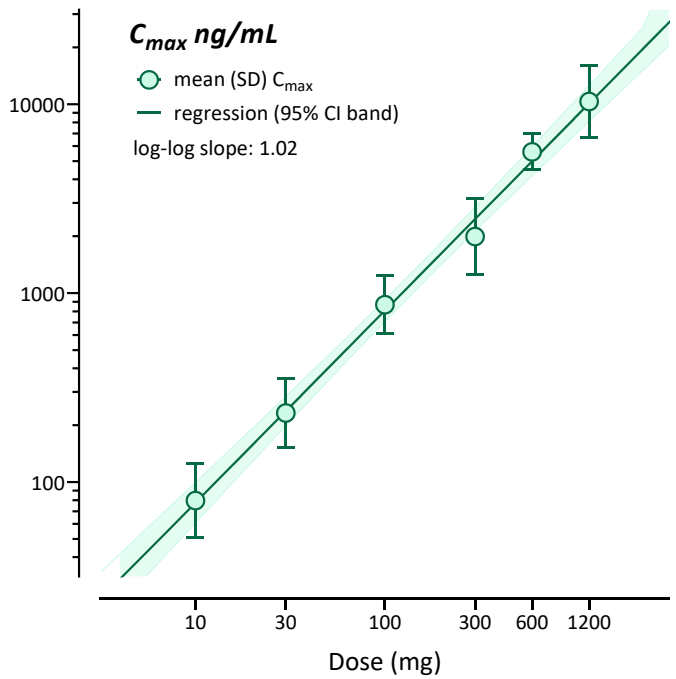
Part 2 – Multiple ascending dose
- Healthy subjects
- PK & PD
- Safety and tolerability
- 12 subjects per cohort
  10:2 active : placebo
- Multiple dose levels
- Ongoing

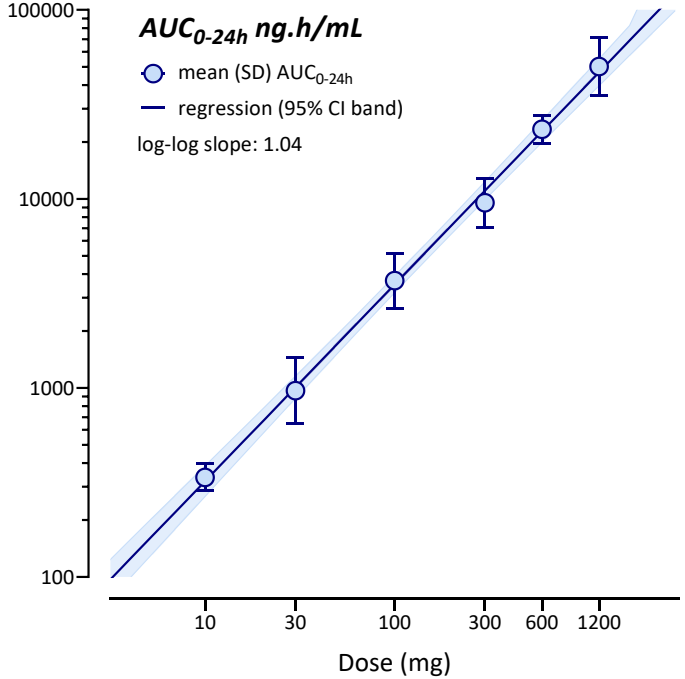
Part 3 – Proof of concept in PNH patients
- Poor responders to eculizumab or ravulizumab, or naïve to treatment
- Up to 16 patients total
- Multiple dose levels

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

Single Dose PK Profile of Oral BCX9930 in Healthy Subjects



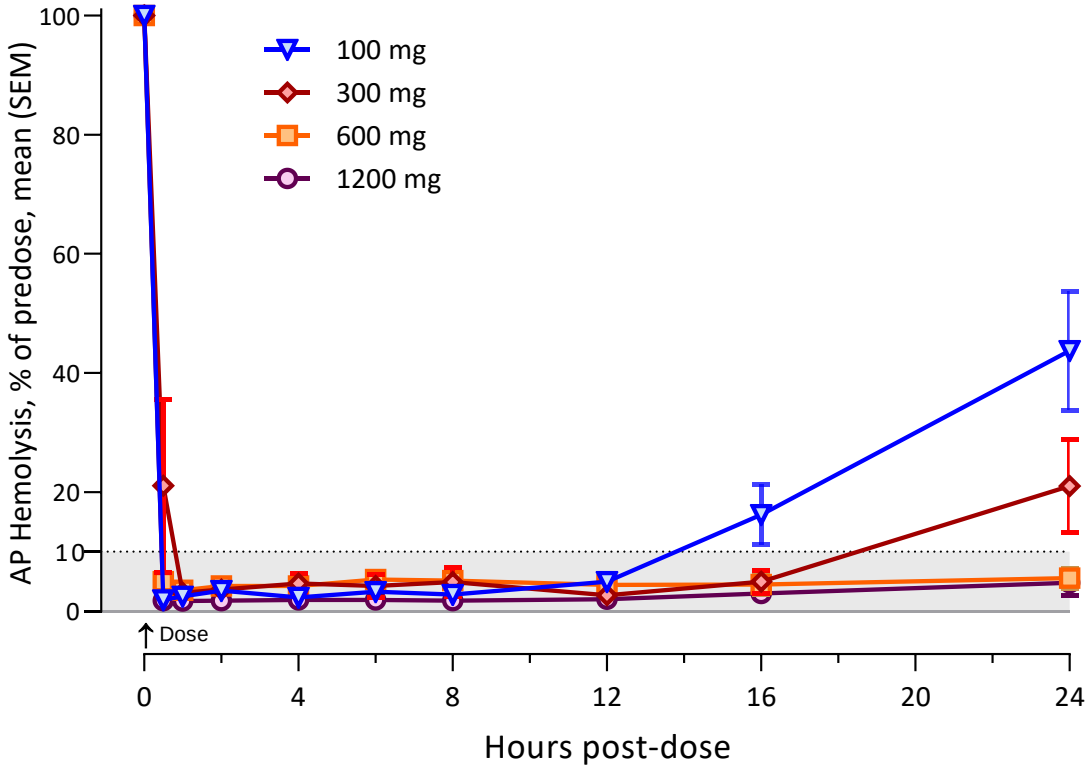




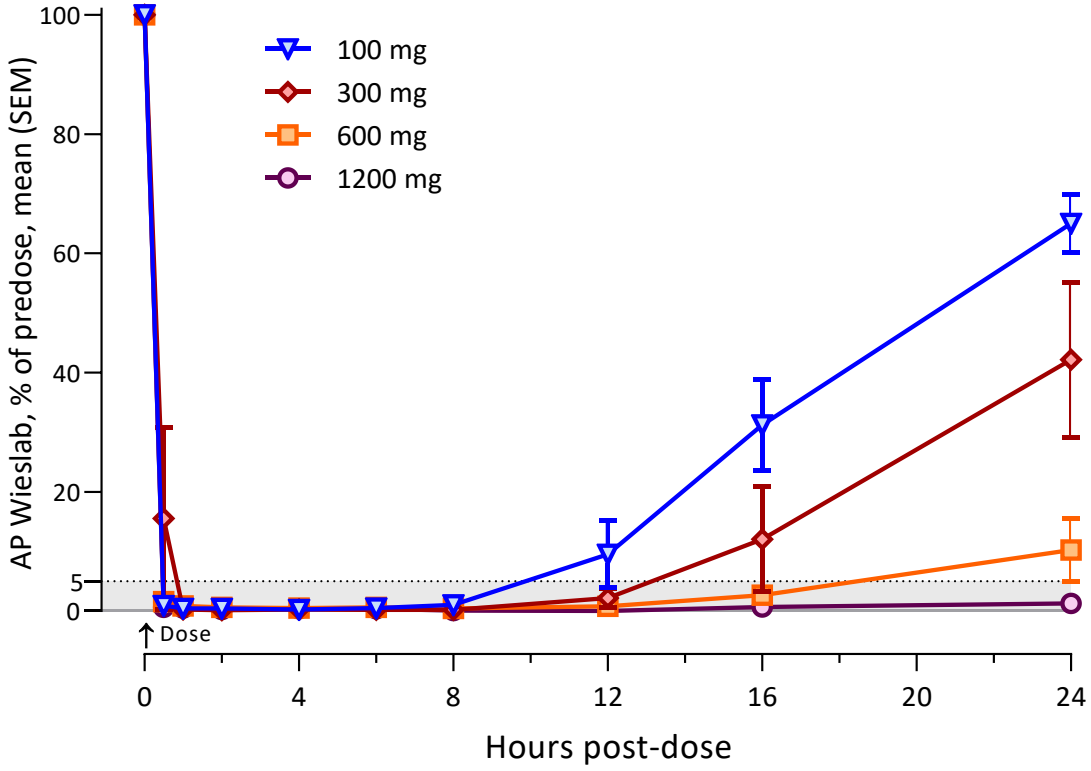
Suppression of AP Activity After Single Oral Doses of BCX9930

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

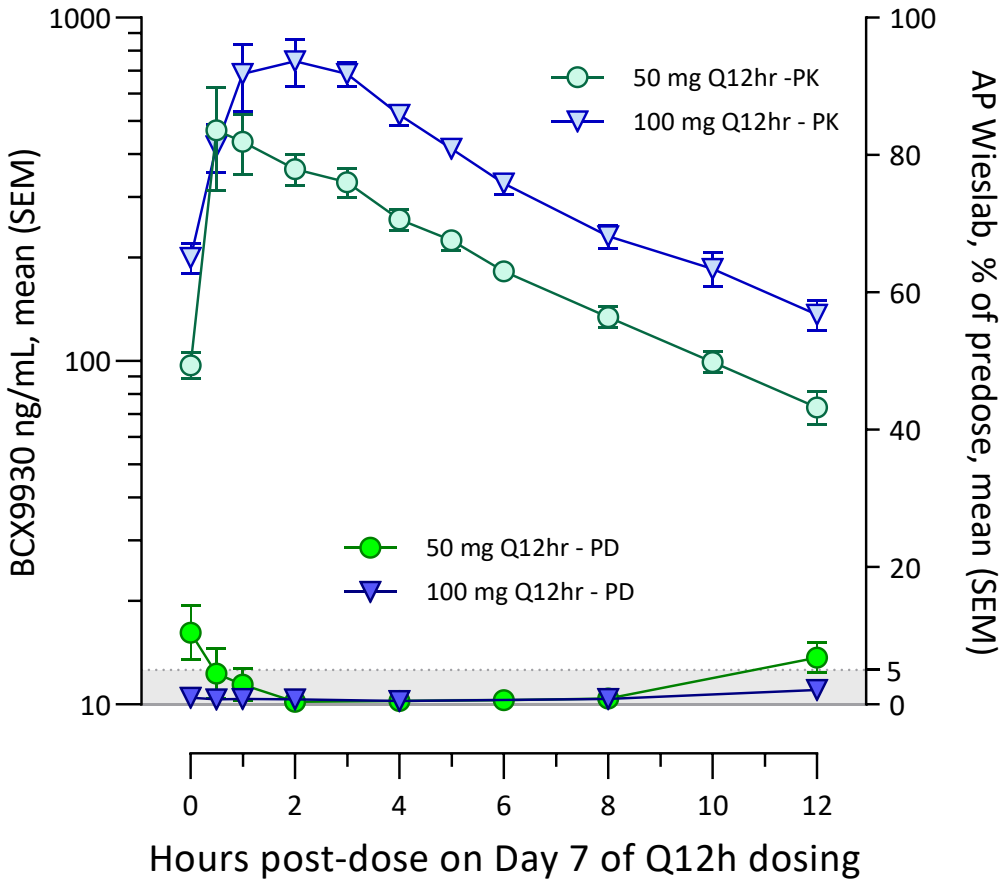
Assay: AP Hemolysis

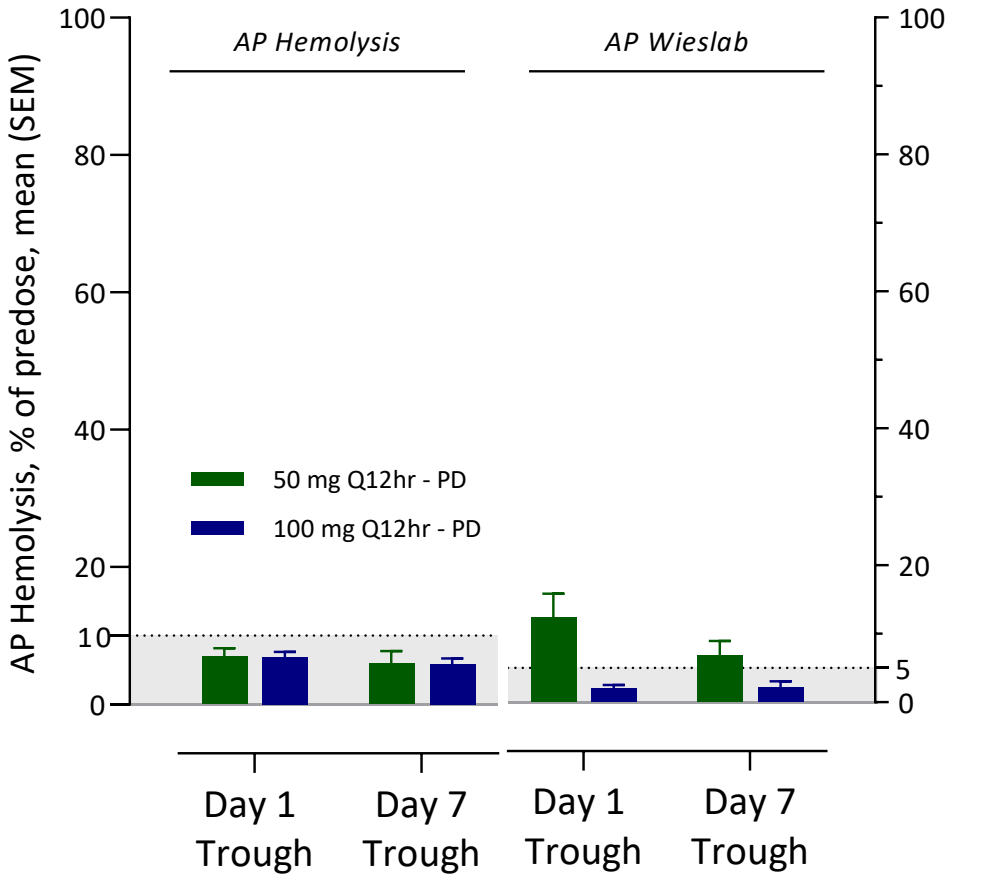


Assay: AP Wieslab



Steady State PK and PD with Q12hr Dosing of BCX9930





BCX9930 Phase 1 Trial: Summary

Safety & Tolerability

- Safe and generally well-tolerated at all doses
- No serious adverse events and no discontinuations
- 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 some MAD subjects that was self-limited and resolved in 4 to 8 days post-onset
  - 2 subjects in 50 mg cohort, 7 subjects in 100 mg cohort

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

Program Advancing to Part 3 of Trial, PoC in PNH Patients

- Will evaluate both poor responders and treatment naïve PNH patients
- Data from PNH PoC expected 1H 2020

Cash Position & 2019 Guidance (in Millions)
Added >$80 M to Balance Sheet in Past 3 Weeks

Cash & investments at December 31, 2018	$128
Cash & investments at September 30, 2019[A]	**$70**
Senior Credit Facility [B]	$50
FY 2019 GUIDANCE	
Operating cash utilization	**$105 – 130**
Operating expenses [C]	**$120 – 145**

A – Does not include $22M upfront payment from Torii for Japanese commercial rights to BCX7353 or $63.3M from November 2019 equity offering (before deducting underwriting discounts and commissions and other estimated offering expenses payable by BioCryst).

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

C - Excludes equity-based compensation. See "Management's Discussion and Analysis of Financial Condition and Results of Operations - Financial Outlook for 2019" in Part I, Item 2 of our Quarterly Report on Form 10-Q for the quarter ended September 30, 2019.

BioCryst: Multiple Meaningful Milestones Anticipated in 2019-2020