May 2021
Corporate Presentation

Forward-Looking Statements

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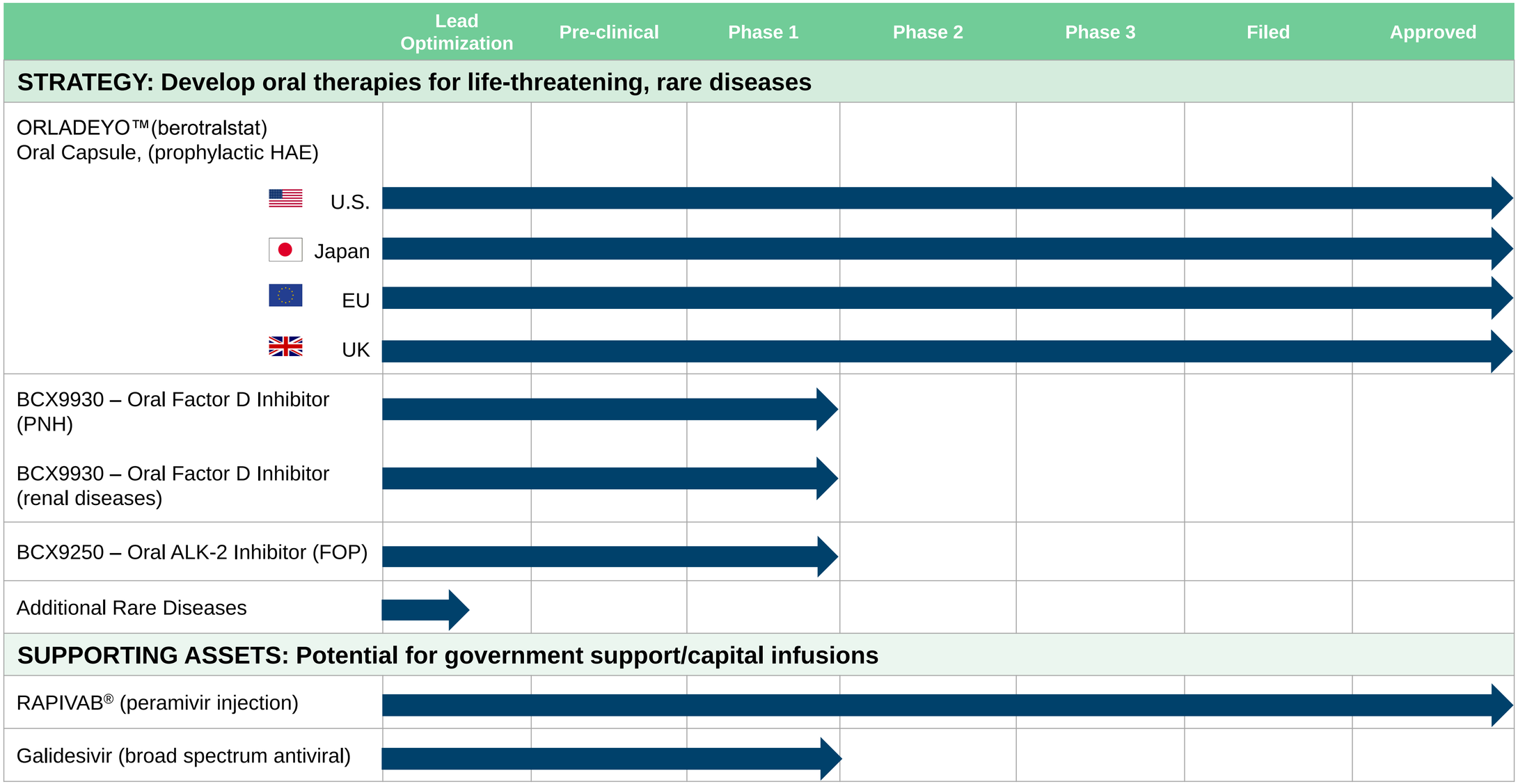
Delivering extraordinary
Empowering ordinary

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

BioCryst's Robust Pipeline



Significant Upcoming Milestones in 2021

Q12021

- **Approval** decision on ORLADEYO in Japan (January 2021)
- **Data** from completed BCX9930 dose ranging study in PNH (R&D Day: March 2021)

Q22021

- **Approval** decision on ORLADEYO in EU
- **Revenues** reported from Q1/first full quarter of ORLADEYO sales in US
- **Launch** of ORLADEYO in Japan
- **Launch** of ORLADEYO in Germany

Q32021

BCX9930 Advanced Development Trials

BCX9250 Next Steps

Q42021

ORLADEYO REVENUES

Strong Start to Launch as HAE Patients Switch to ORLADEYO™

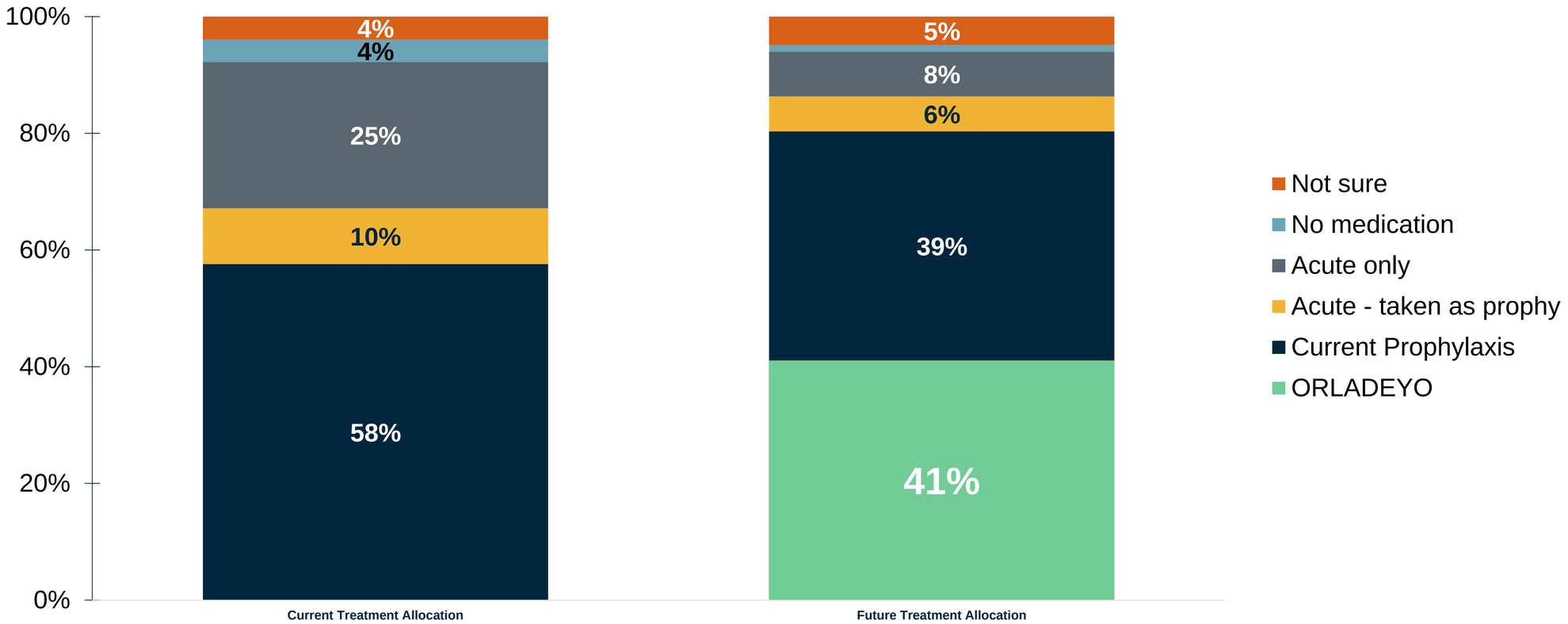
orladeyo™
(berotralstat) capsules 150 mg

- **$10.9 million in Q1 2021 net ORLADEYO revenue**
  - Majority of Q1 2021 ORLADEYO revenue came from new patients switching to ORLADEYO from either injectable/infused prophylactic medications or from acute-only treatment
  - Remainder from patients transitioning from clinical trials and the early access program
  - Significant expansion of prescriber base beyond investigators
  - Expect majority of patients to have access to coverage for ORLADEYO by mid-year
  - Q1 results consistent with market research and clinical experience showing desire to switch to ORLADEYO

Physicians Expect to Prescribe ORLADEYO 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 ORLADEYO Available – Mean % of Patients

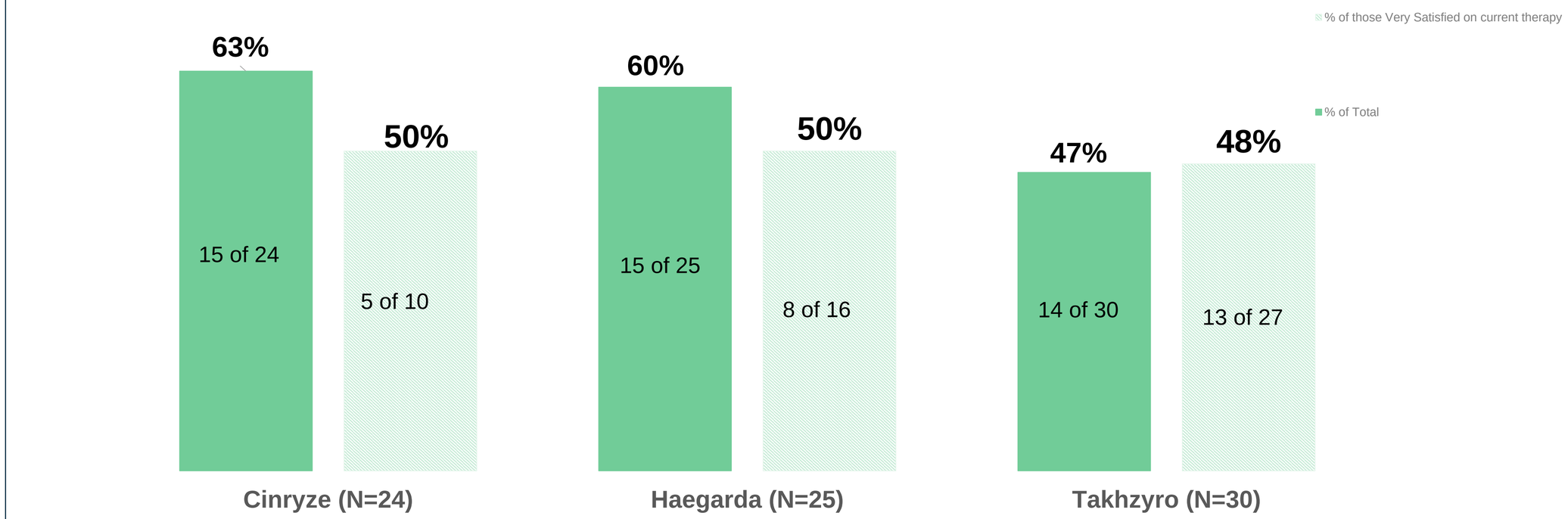


All Qualified Respondents (n=175)

Source: BioCryst Proprietary Market Research Study, 2019. Respondents were shown a blinded profile (Treatment X) of ORLADEYO, 24-week results of APeX-2, Physicians were asked to perform a patient allocation.

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

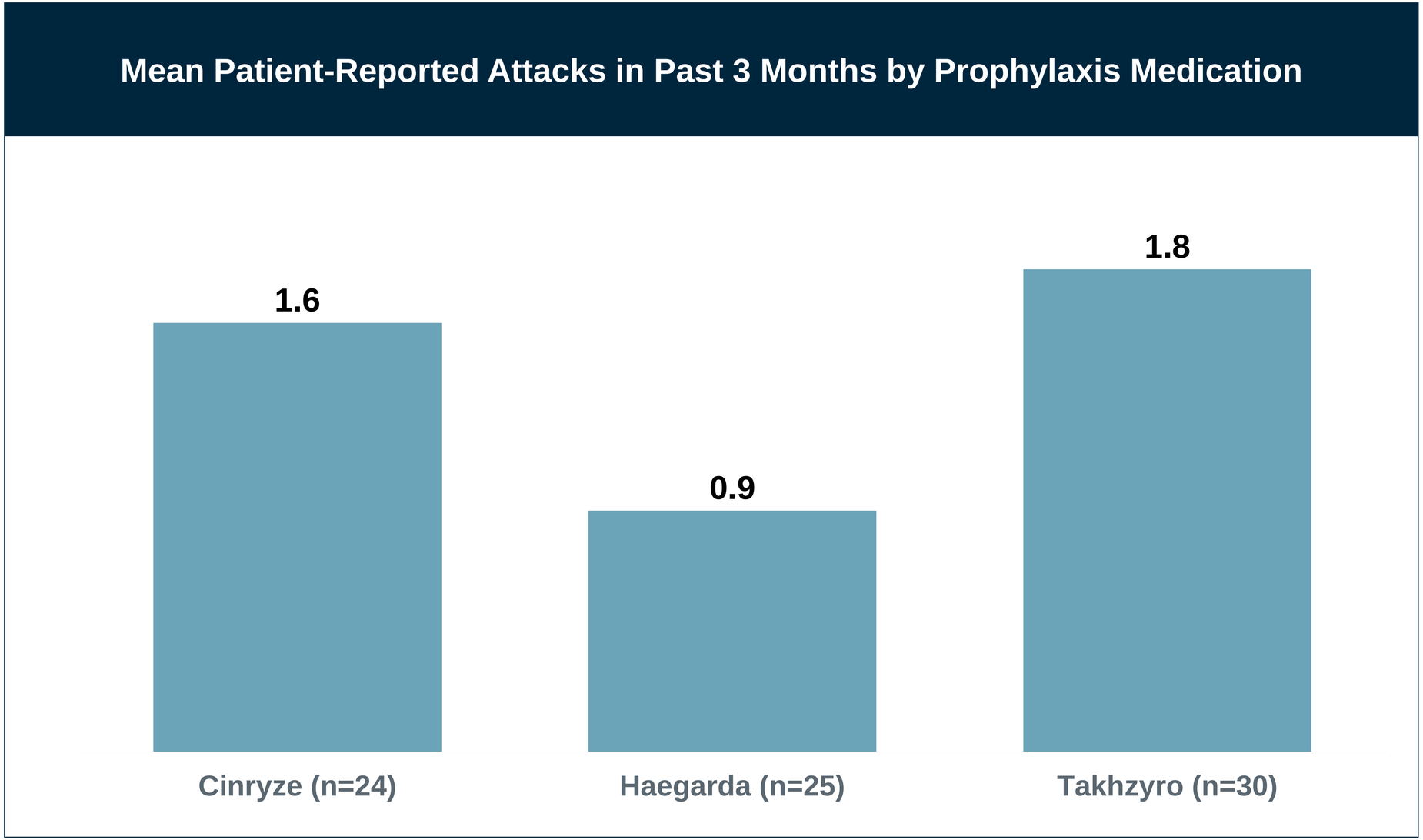
Prophylaxis Patients VERY WILLING to Use BCX7353



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

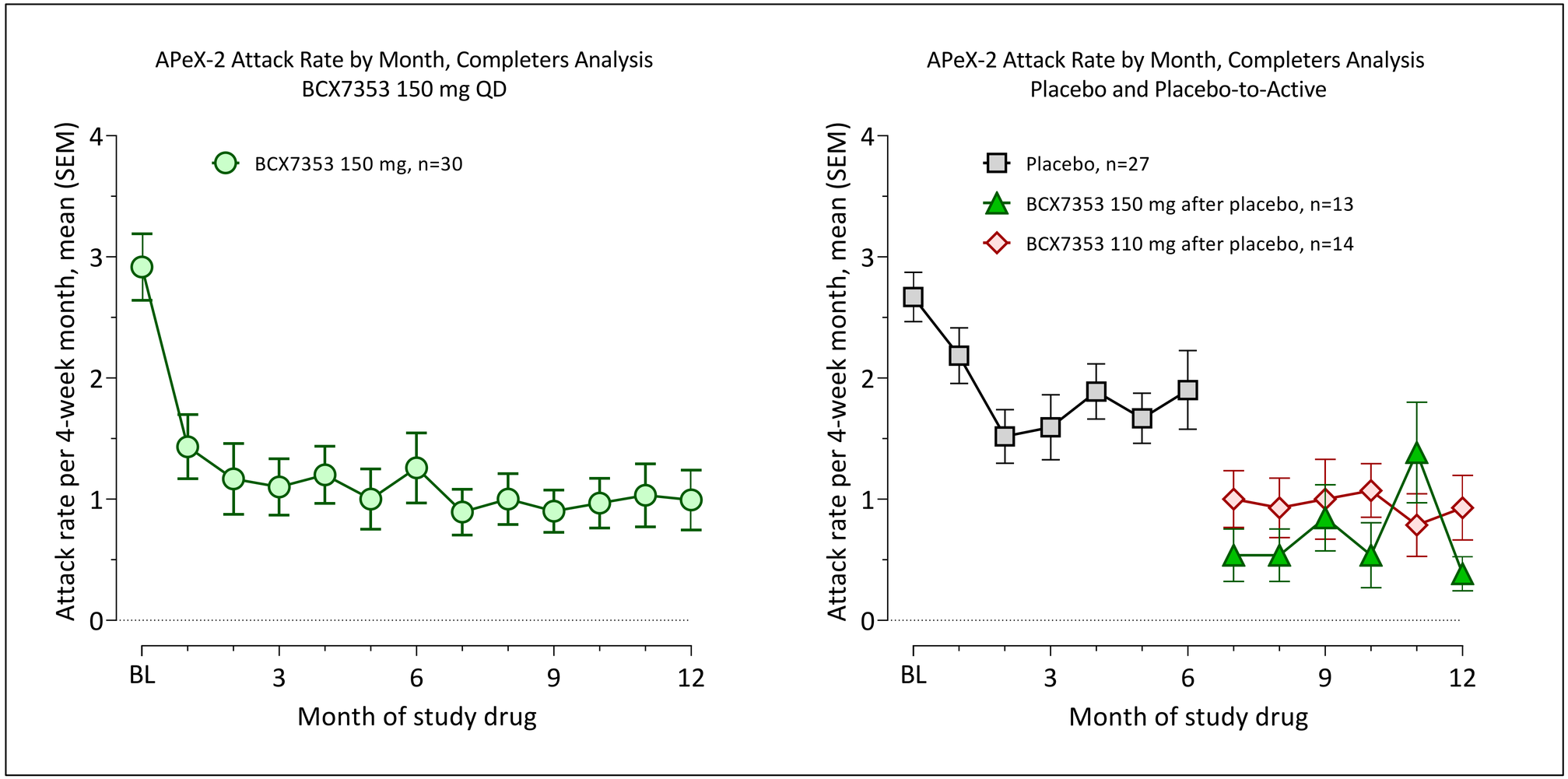
Patients Report Breakthrough Attacks with Injectable/Infused Treatments



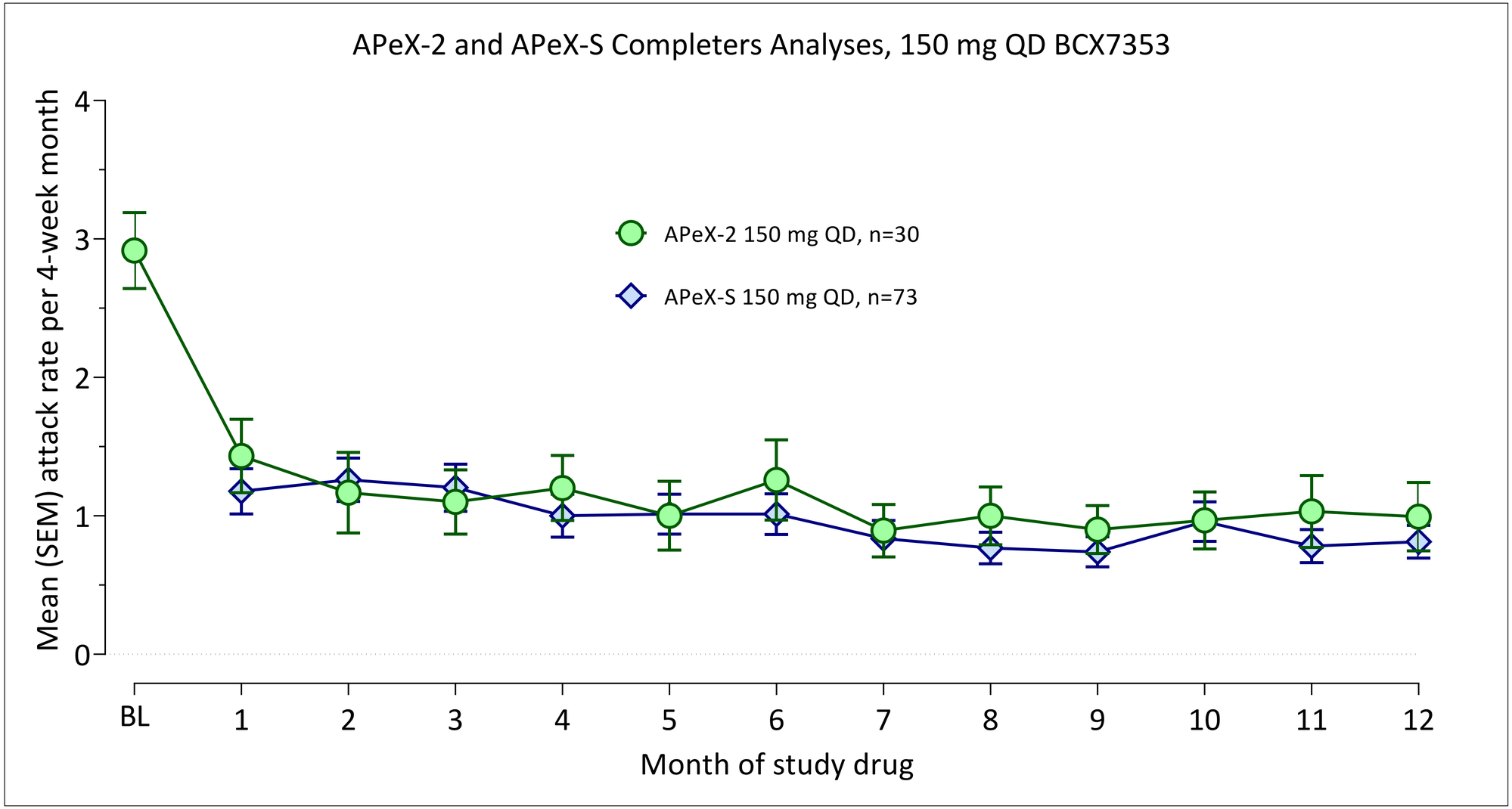
Currently Taking Medication Prophylactically

Source: BioCryst Proprietary Market Research Study, 2019.

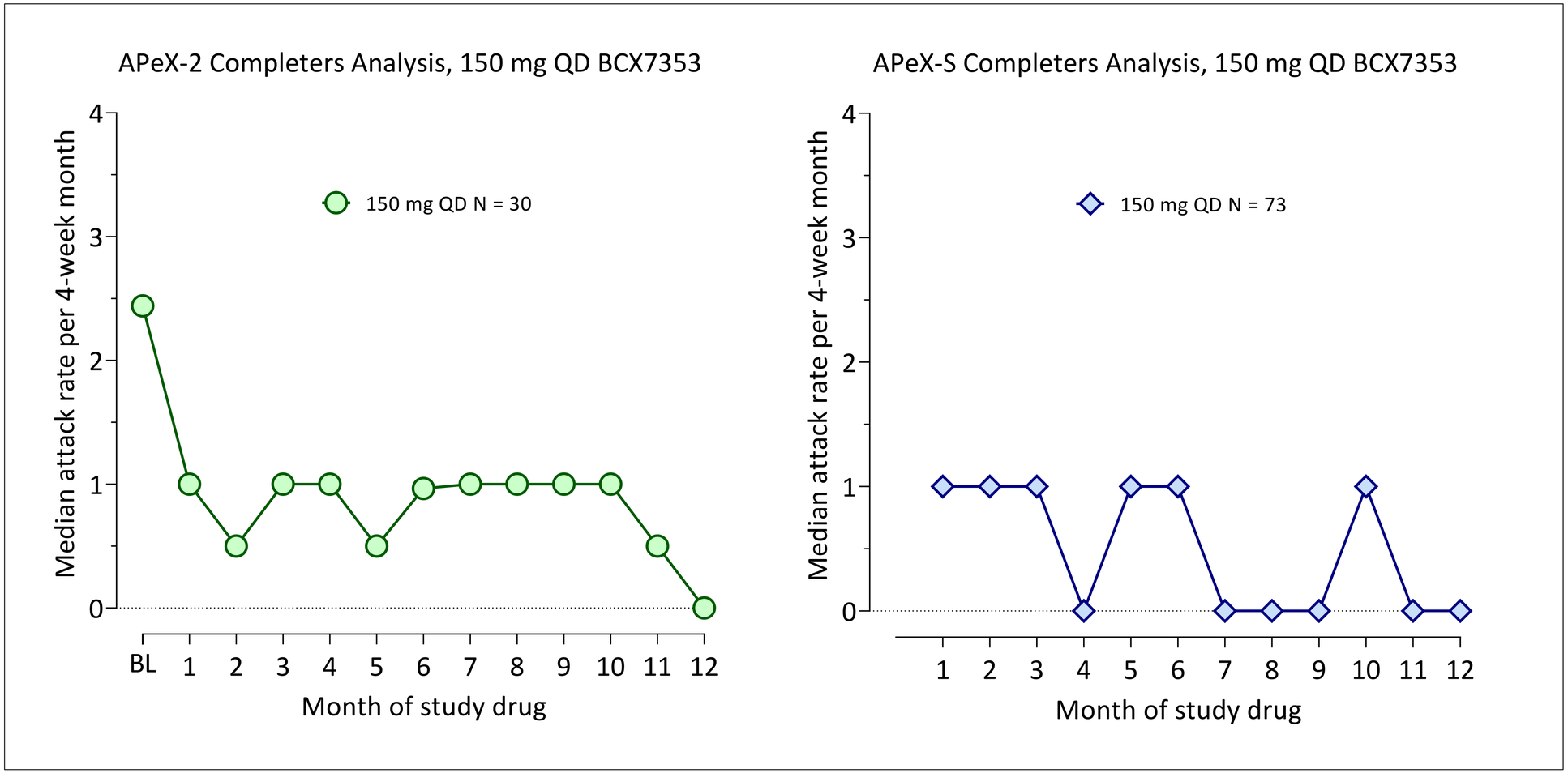
Rapid and Sustained HAE Attack Rate Reduction: Analyses for APeX-2: 150 mg and Placebo 48-week Completers



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



Approved Label: ORLADEYO™ (berotralstat) Safety

In APeX-2 (part 1), the most common[a] treatment-emergent adverse reactions were abdominal pain, vomiting, diarrhea, back pain, and gastroesophageal reflux disease (GERD)

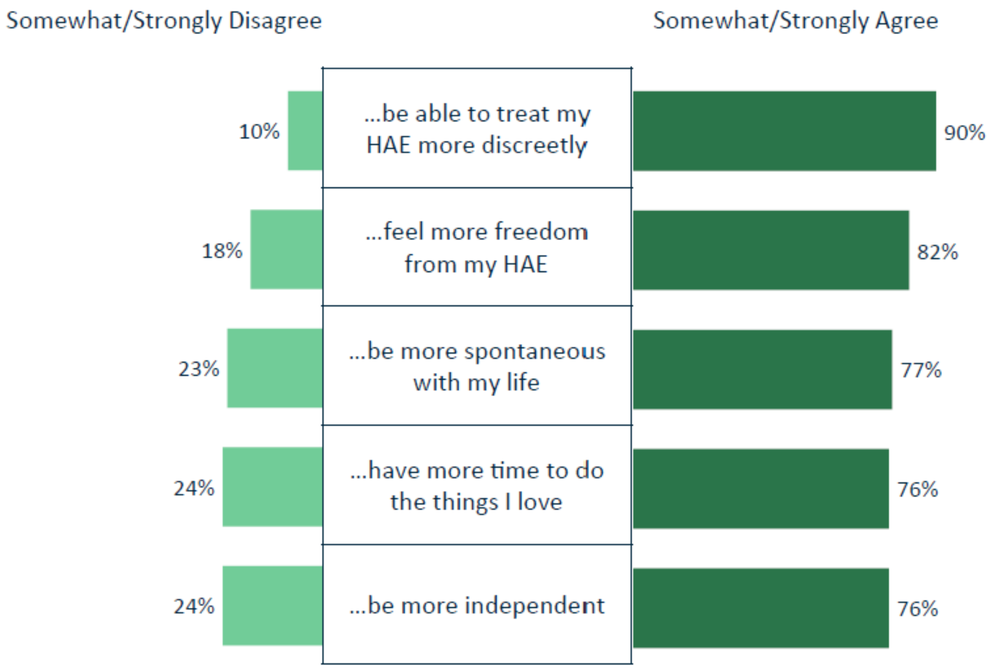
Adverse reactions	Placebo (n=39)	ORLADEYO 110 mg (n=41)	ORLADEYO 150 mg (n=40)
	n (%)	n (%)	n (%)
Abdominal pain[b]	4 (10)	4 (10)	9 (23)
Vomiting	1 (3)	4 (10)	6 (15)
Diarrhea[c]	0	4 (10)	6 (15)
Back pain	1 (3)	1 (2)	4 (10)
GERD	0	4 (10)	2 (5)

[a]≥10% and higher than placebo. [b]Includes abdominal pain, abdominal discomfort, abdominal tenderness, and upper abdominal pain. [c]Includes diarrhea and frequent bowel movements.

Findings from the open-label, long-term safety study, APeX-S (interim safety population, n=227), support the data observed in APeX-2 (part 1)

Significant Burden of Treatment Reported by Patients, Caregivers, and Treating Physicians

Patient perspective:
If I were prescribed a once daily pill to prevent (prophylaxis) HAE attacks, I would…*

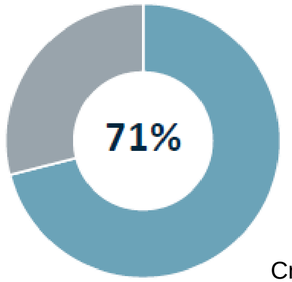


Agreement with statements regarding a once-daily oral HAE medication (n = 75)

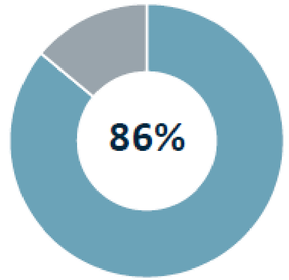
Radojicic et al., ACAAI 2020, Poster #160

Caregivers perspective: How patients perceive their prophylactic treatment

[Patient] is tired of his/her infusions/injections
*% of caregivers who somewhat/strongly agree**

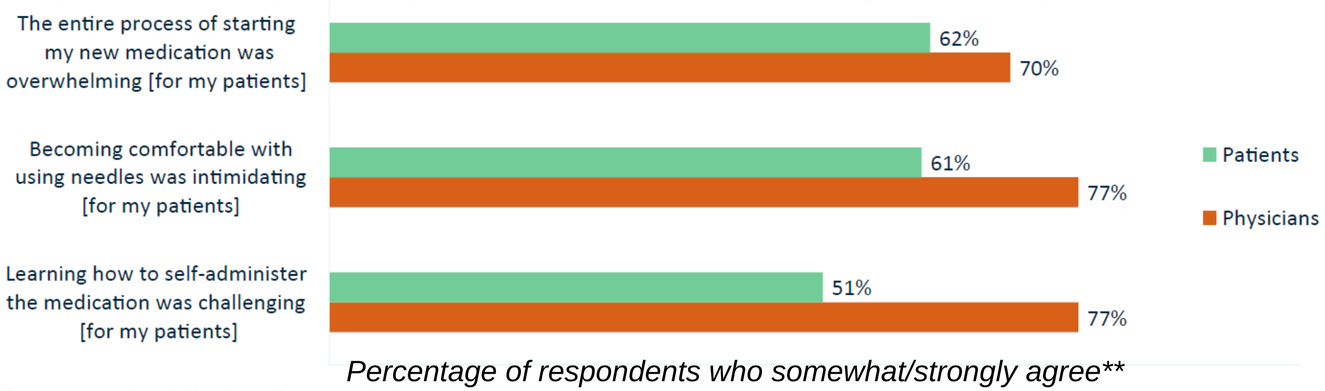


[Patient] is satisfied with his/her current HAE treatment, but would still be interested in one that is easier to administer
*% of caregivers who somewhat/strongly agree**



Craig et al., ACAAI 2020, Poster #161

Physician and patient perceptions about starting HAE prophylaxis:



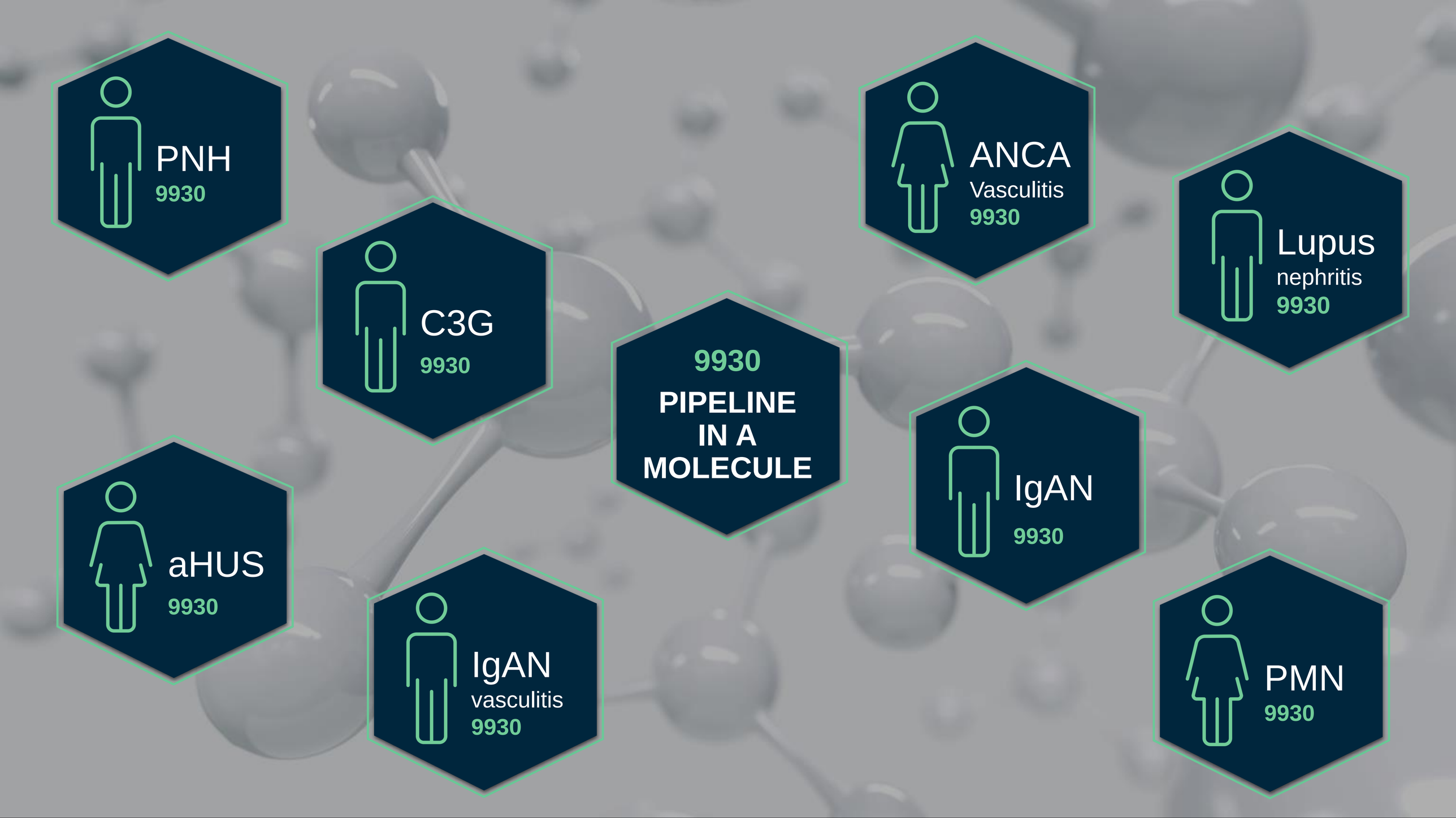
*Percentage of respondents who somewhat/strongly agree***

Riedl et al., ACAAI 2020, Poster #162

Cross-sectional study conducted via three double-blinded surveys with HAE patients (n=75), caregivers (n=30) and physicians (n=109)

**Bases vary; not applicable/not sure excluded;*

***Bases vary; not applicable/not sure excluded; text in brackets was included in the response options for physicians*


PNH
9930
C3G
9930
9930
PIPELINE
IN A
MOLECULE
aHUS
9930
IgAN
vasculitis
9930
ANCA
Vasculitis
9930
IgAN
9930
Lupus
nephritis
9930
PMN
9930

Alternative Complement Pathway Activation Products in Urine and Kidneys of Patients with ANCA-Associated GN

Shen-Ju Gou, Jun Yuan, Chen Wang, Ming-Hui Zhao, and Min Chen

Clin J Am Soc Nephrol 8: 1884–1891, 2013. doi: 10.2215/CJN.02790313

Complement activation products in the circulation and urine of primary membranous nephropathy

Mu-fan Zhang[1,2,3,4], Jing Huang[1,2,3,4], Yi-miao Zhang[1,2,3,4], Zhen Qu[1,2,3,4], Xin Wang[1,2,3,4], Fang Wang[1,2,3,4], Li-qiang Meng[1,2,3,4], Xu-yang Cheng[1,2,3,4], Zhao Cui[1,2,3,4*], Gang Liu[1,2,3,4] and Ming-hui Zhao[1,2,3,4,5]

BMC Nephrology (2019) 20:313

The Emerging Role of Complement Proteins as a Target for Therapy of IgA Nephropathy

Dana V. Rizk[1], Nicolas Maillard[2], Bruce A. Julian[1], Barbora Knoppova[3,4], Todd J. Green[3], Jan Novak[3] and Robert J. Wyatt[5]**

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*

Complement Alternative Pathway's Activation in Patients With Lupus Nephritis

Di Song, PhD, Wei-yi Guo, PhD, Feng-mei Wang, PhD, Yong-zhe Li, PhD, Yan Song, MD, Feng Yu, MD and Ming-hui Zhao, MD, PhD

Am JMedSci 2017;353(3):247–257

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)

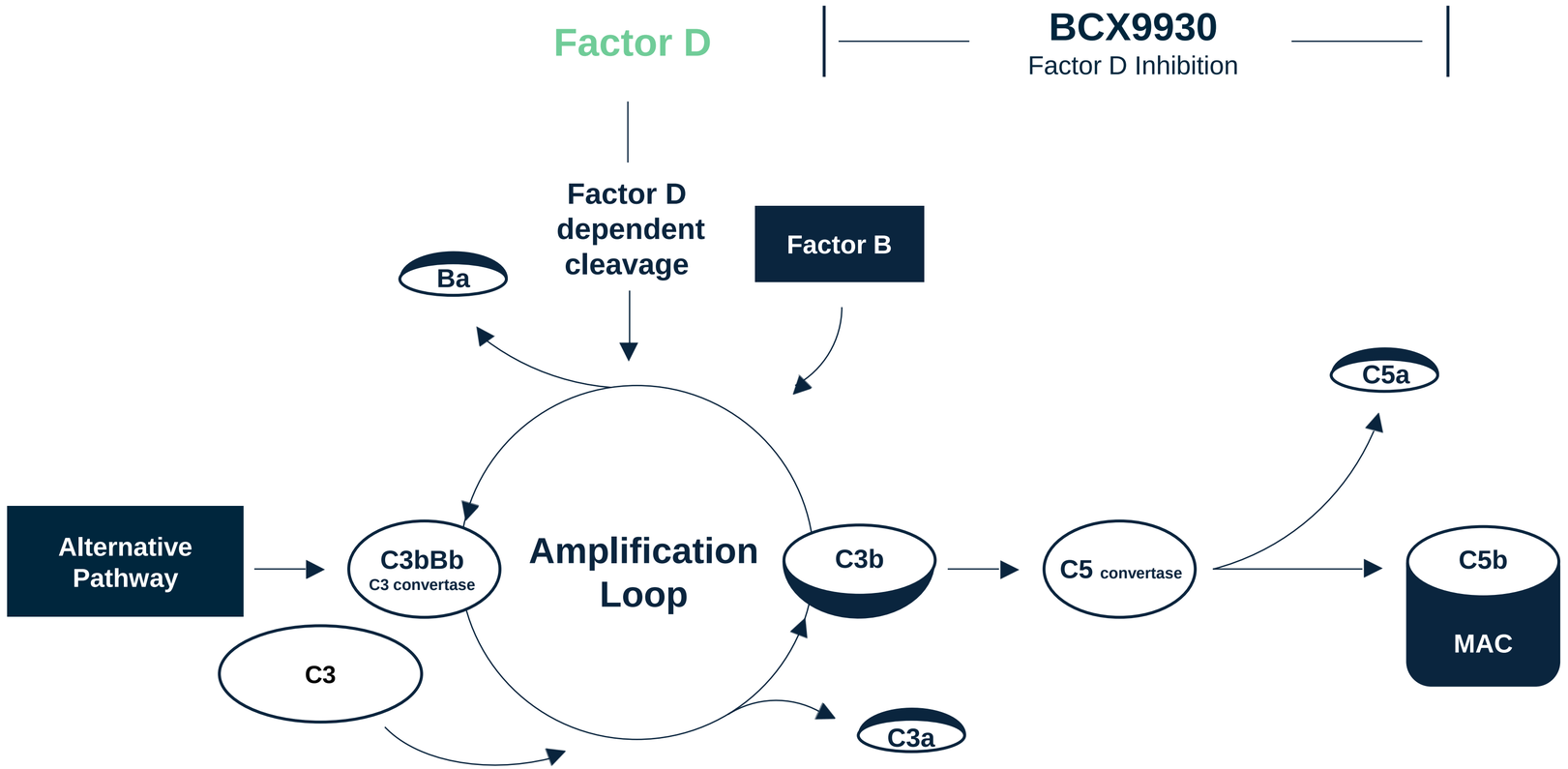
Sanjeev Sethi, MD, PhD[1] Fernando C. Fervenza, MD, PhD[2]

C3 glomerulopathy — understanding a rare complement-driven renal disease

Richard J. H. Smith[1], Gerald B. Appel[2], Anna M. Blom[3], H. Terence Cook[4], Vivette D D'Agati[5], Fadi Fakhouri[6], Véronique Fremeaux-Bacchi[7], Mihály Józsi[8], David Kavanagh[9], John D. Lambris[10], Marina Noris[11], Matthew C. Pickering[12], Giuseppe Remuzzi[11,13,14], Santiago Rodriguez de Córdoba[15], Sanjeev Sethi[16], Johan Van der Vlag[17], Peter F. Zipfel[18,19] and Carla M. Nester[1]*

Factor D:
An Outstanding Drug Target for Complement-mediated Diseases

- Factor D is essential to initiate the Alternative Pathway
- Blocking Factor D blocks the Alternative Pathway and all downstream products



Spectrum of Alternative Pathway Dysregulation Diseases

HEMATOLOGY

PNH
paroxysmal nocturnal hemoglobinuria

aHUS
atypical hemolytic uremic syndrome

RHEUMATOLOGY

ANCA vasculitis
antineutrophil cytoplasmic antibody-associated vasculitis

Lupus nephritis

IgAN vasculitis

NEPHROLOGY

C3G
glomerulonephritis

PMN
primary membranous nephropathy

IgAN
IgA nephropathy

Oral Monotherapy w/BCX9930 Offers Advantage of Controlling Both Intravascular (IVH) and Extravascular (EVH) Hemolysis

Goal of Total Hemolytic Control

IVH Control

↓ LDH

+

EVH Control

↓ Opsonized RBCs

Hemolytic Control

↑ RBC/Granulocyte Clone Size

Patient Outcomes from Controlled Hemolysis over Time

Relieve Anemia

↑ Hemoglobin

Reduce PNH Clinical Symptoms

↓ Appearance of Symptoms

Avoid Transfusions

↓ Transfusions

A. M. Risitano *et al.*, *Front Immunol* **10**, 1157 (2019)

PNH Proof of Concept Study Design
Study Goals and Patient Eligibility Criteria

Key Study Goals

- Evaluate safety and tolerability
- Identify therapeutically active dose regimen
- Characterize PD on clinical outcomes & biomarkers
- Characterize PK

Key Eligibility Criteria at Screening:

All Patients:

- PNH clone size > 10%
- Platelet count > 30,000/µL
- Reticulocyte count > 100,000/µL

Naïve Criteria:

- No C5 Inhibitor
- Hb < 10 g/dL or blood transfusion within the last 12 months
- LDH ≥ 2 x ULN

Inadequate Responders Criteria:

- Stable C5 Inhibitor for 6 months
- Hb < 10 g/dL or blood transfusion within the last 3 months

PNH Proof of Concept Study Design

Patients Enrolled

Cohort	Naive	Inadequate Responders
1	4	--
2	4	2
3	2	4
Clinical Site Locations	*South Africa*	*United Kingdom Austria*

Dose Escalation per Cohort

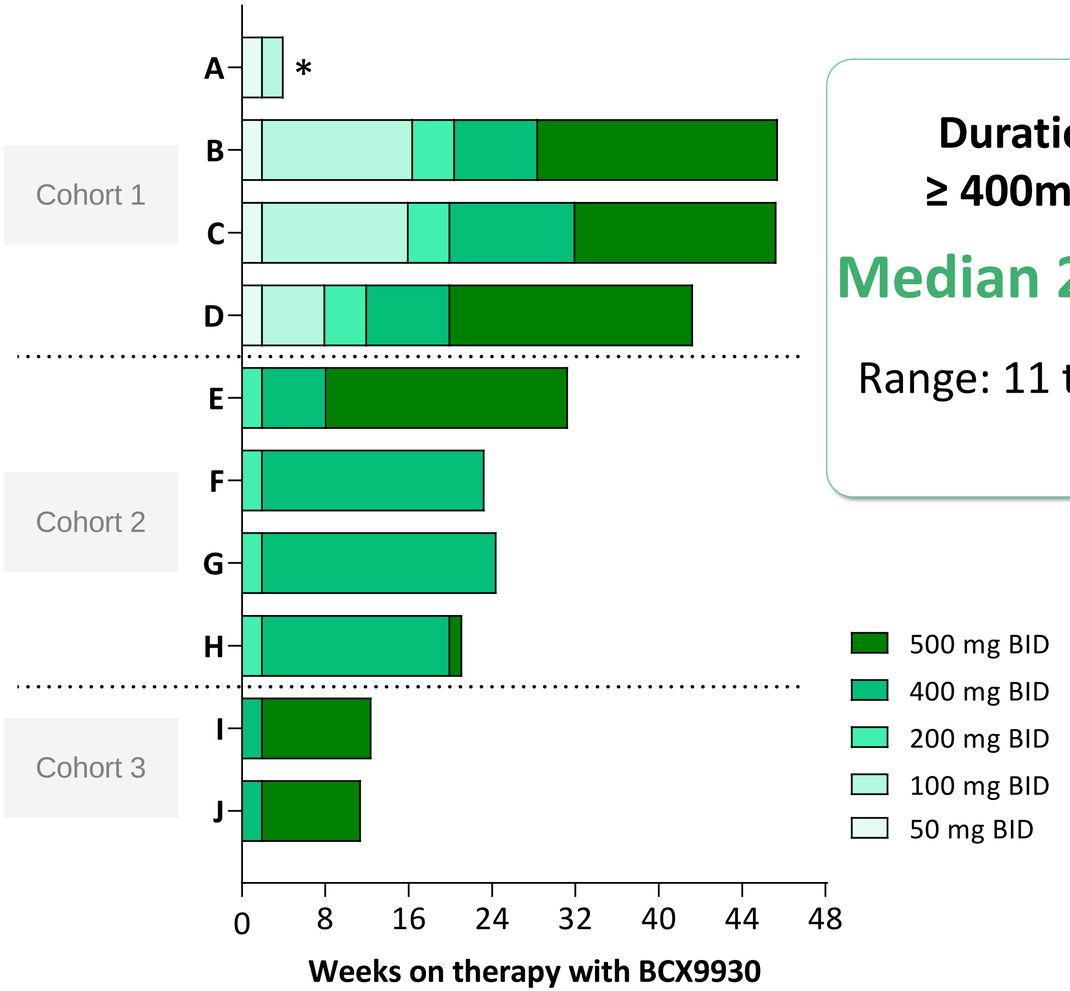
Days 1 - 14	Days 15 - 28	Extension > 28 days
50 mg BID	100 mg BID	Patients **benefitting on treatment may continue** on BCX9930 and **dose-escalate** at physicians' discretion
200 mg BID	400 mg BID	
400 mg BID	500 mg BID	

- 10 Naïve patients enrolled w/ BCX9930 monotherapy treatment
- 6 Inadequate Response patients enrolled w/ BCX9930 + C5-inhibitor treatment

Naïve Patients Enrolled had Severe Disease Prior to Treatment

9 naive patients remain in study with average overall treatment duration of 6 months

Parameter @ Baseline	N = 9 *
Age	29.2 years
Duration since Diagnosis	3.2 years
Gender	8 (89%) Male
Race: African Caucasian Other	 5 (56%) 3 (33%) 1 (11%)
Bone Marrow Failure	4 (44%)
RBC transfusion-dependent (prior 12 months)	7 (78%)
RBC transfusions in prior 12 months, mean	7.6 units
Hb, mean g/dL (range)	8.3 (6 – 11)
Reticulocyte count, mean $10^3/\mu L$ (range)	176 (104 – 305)
LDH, mean x ULN (range)	7.5 (3.7 – 13.1)
AST, mean x ULN (range)	2.1 (0.8 – 3.8)



Duration on ≥ 400mg BID:

Median 22 weeks

Range: 11 to 31 weeks

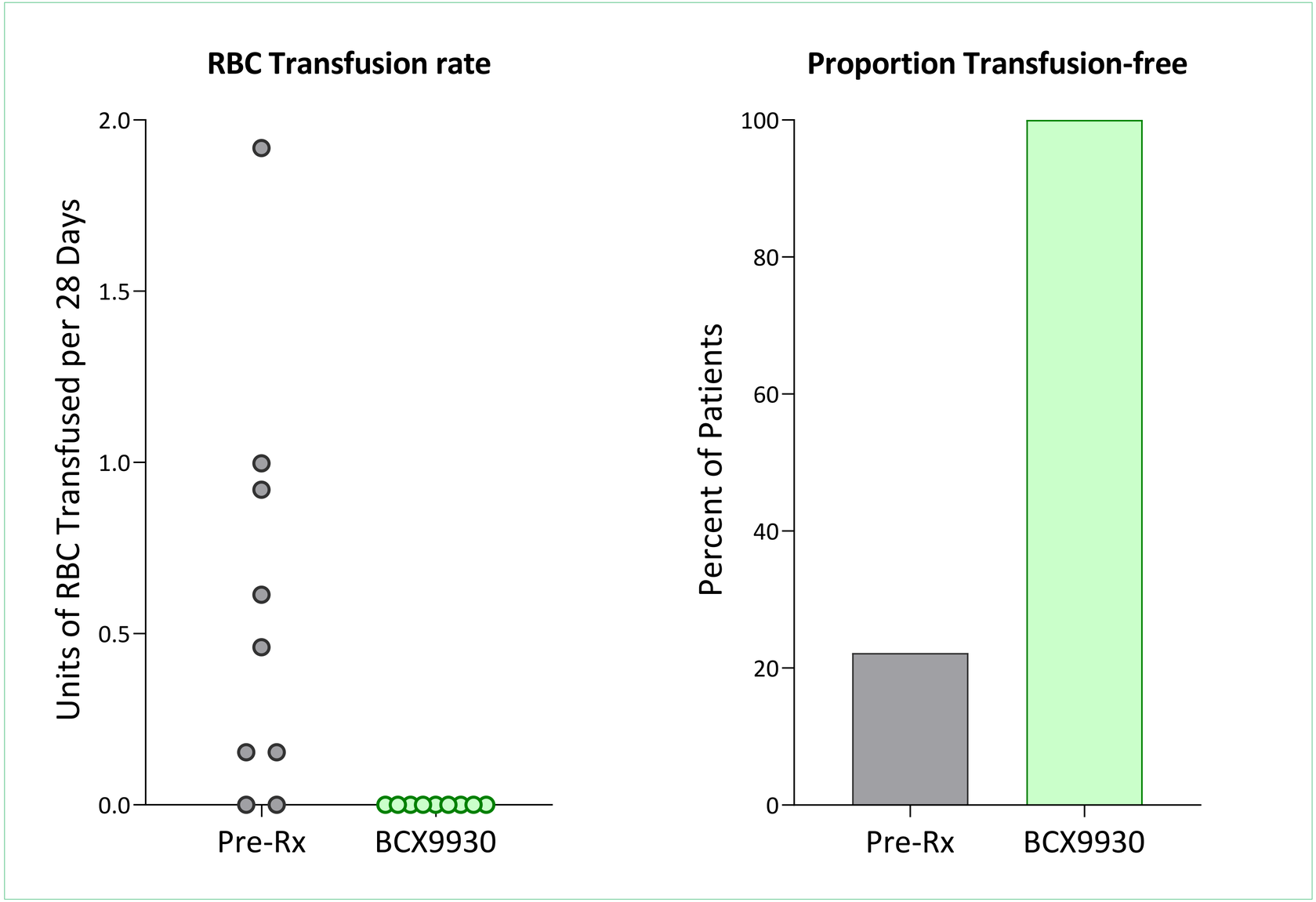
∗ *As disclosed in May 2020, Patient A discontinued due to an unrelated SAE.*

C5-inhibitor Naïve Patients had Significant Increase in Hemoglobin as a Result of Control of Hemolysis, with Reductions in Reticulocytes, LDH, Bilirubin, and AST

Response Parameter	Baseline N = 9	Last visit* N = 9	Change from Baseline
Hemoglobin g/dL, mean (SEM)	8.3 (0.6)	11.8 (0.6)	+3.5 (0.6)
Hemoglobin > 12 g/dL, n (%)	0	5 (56%)	+5 (56%)
Hemoglobin > 10 g/dL, n (%)	2 (22%)	7 (78%)	+5 (56%)
RBC clone size %, mean (SEM)	47% (5)	86% (4.2)	+40% (4.7)
Reticulocytes 10^3/µL, mean (SEM)	176 (21)	115 (8.5)	-60 (21)
Patients with reticulocytes ≤ 150,000/µL, n (%)	4 (44%)	8 (89%)	+4 (44%)
LDH U/L, mean (SEM)	1721 (264)	544 (104)	-1177 (281)
LDH xULN, mean (SEM)	7.5 (1.2)	2.0 (0.3)	-5.5 (1.1)
Total bilirubin mg/dL, mean (SEM)	1.24 (0.19)	0.59 (0.05)	-0.66 (0.19)
AST U/L, mean (SEM)	86 (14)	22 (4.8)	-64 (14)

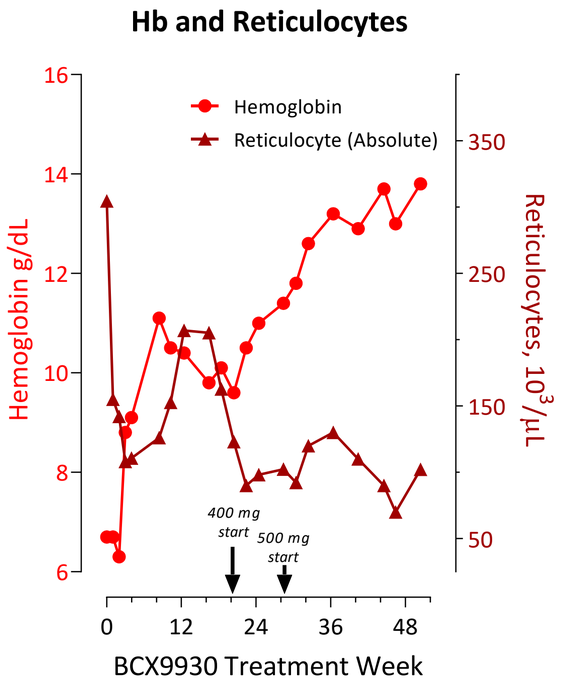
Last treatment visit at 400mg or 500mg BID

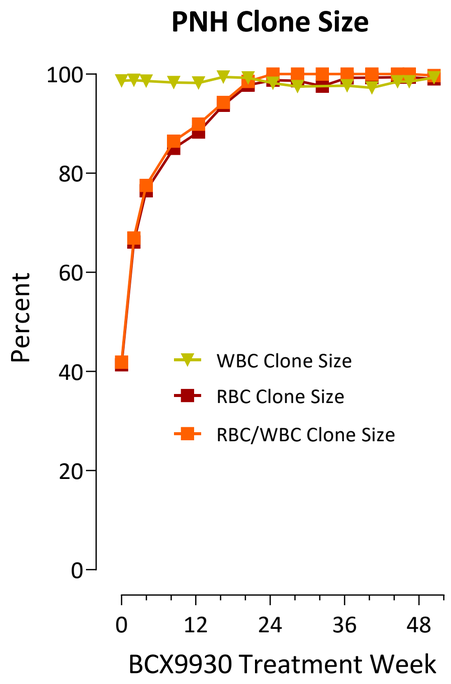
Transfusion Burden in C5-inhibitor Naïve Subjects was Reduced to Zero at Doses of 400 mg or 500 mg BID – 100% of Patients were Free of Transfusions

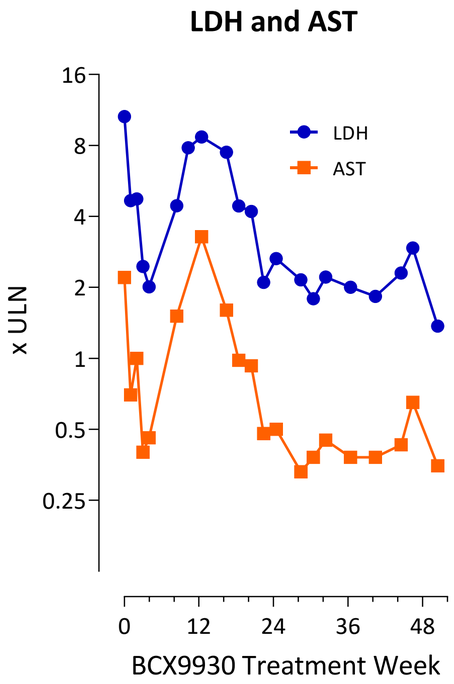


Example of C5-inhibitor Naïve Subject with Dose Escalated to 500 mg BID at Week 28

Patient B – 2 weeks Rx at 50 mg BID then escalating in steps to 500 mg BID







Prior to study, patient received 13 units of RBCs in prior 12 months

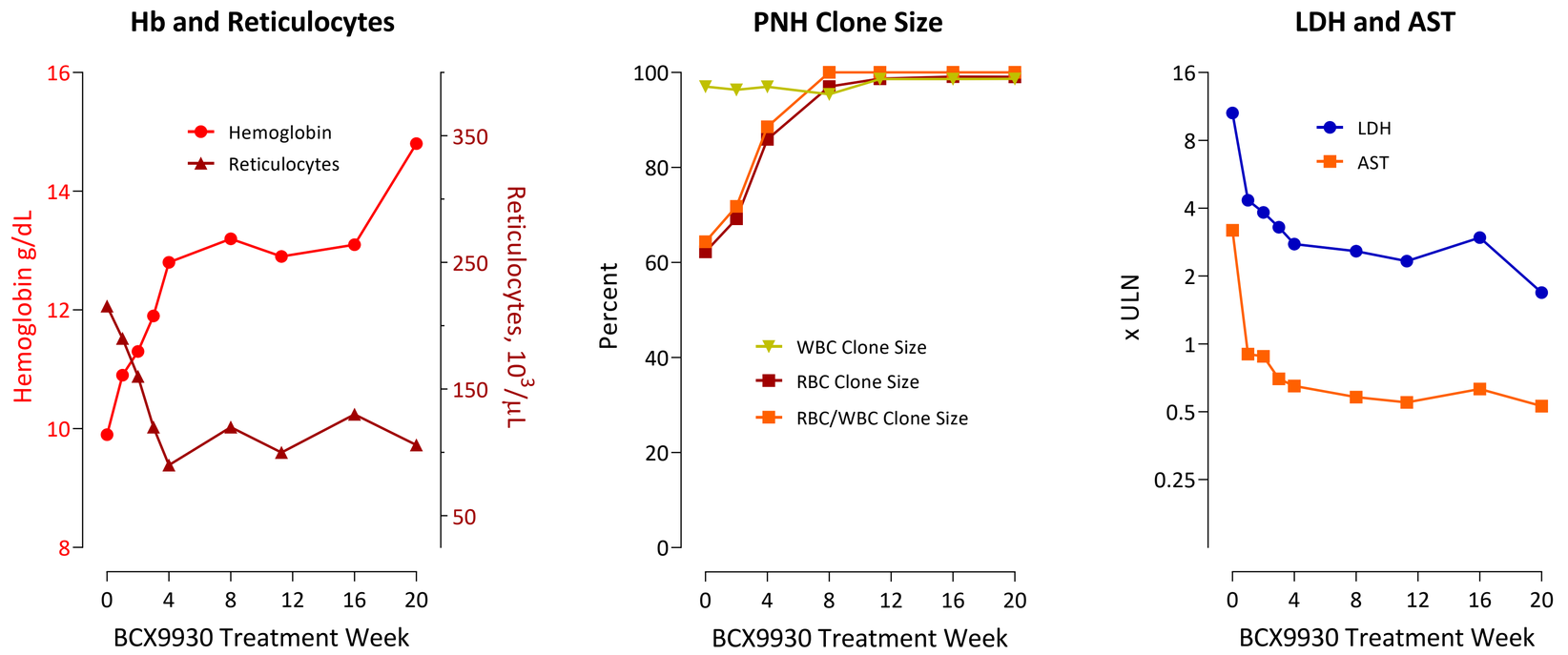
- 2 unit RBC transfusion on study day 15 after 2 weeks of 50 mg BID BCX9930
- Hb rose steadily from 6.7 to 13.8 g/dL over 50 weeks
- Reticulocytes normalized, falling from 305 to 102 x10^3/µL
- RBC/WBC clone size rose from 41.9% to 99.7%
- LDH fell from 10.6 x ULN to 1.4 x ULN at week 50
- AST fell from 2.2 x ULN to 0.35 x ULN
- Tonsillitis was noted in week 46, associated with asymptomatic increase in LDH (<50%) and fall in Hb (<1g/dL)

- *Updated from last September with longer follow up, almost a year of treatment*
- *At suboptimal doses, hemolysis was not controlled*
- *Relative RBC clone size reached >99% – maximum control, then LDH stabilized*
- *Fluctuations in biomarkers occur, for example due to infection – not clinically significant*

Preliminary data

Example of C5-inhibitor Naïve Subject Response Over Time

Patient H – 2 weeks Rx at 200 mg BID then 400 mg BID through week 20



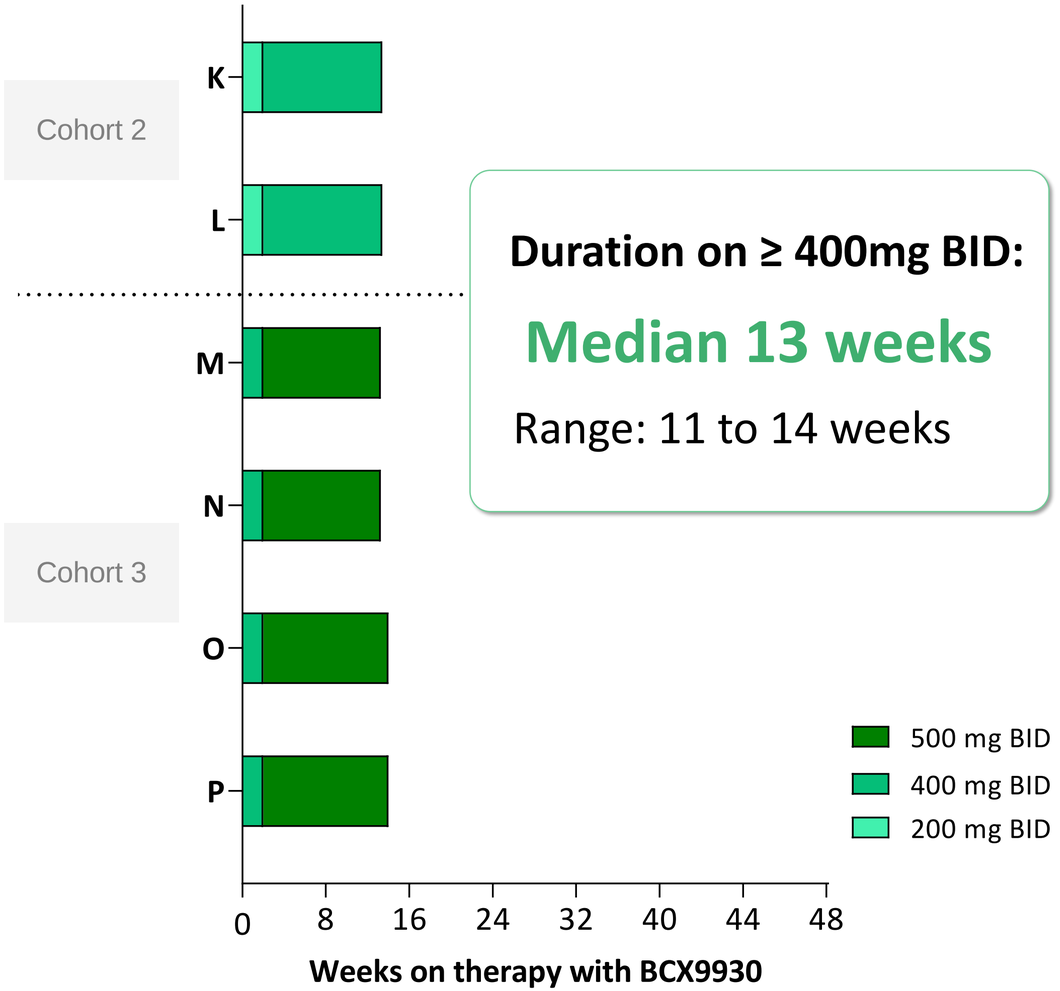
Prior to study, patient received 8 units of RBCs in previous 12 months

- No RBC transfusions on study
- Hb rose from 9.9 to 14.8 g/dL over 20 weeks
- Reticulocytes normalized, falling from 215 to 106 x 10^3/µL
- RBC/WBC clone size rose from 64% to 100%
- LDH fell from 10.6 x ULN to 1.7 x ULN
- AST fell from 3.2 x ULN to 0.5 x ULN

- *Rapid response was seen*
- *The response was durable and improved over time*
- *Relative RBC clone size reached 100% – this means maximum complement inhibition effect*
- *LDH took time to stabilize once effect on clone size was achieved*

Patients with Inadequate Response Represented a Severely Ill Patient Group with Persistent Anemia and Transfusion Dependence Despite Optimized C5 Inhibitor Treatment

Parameter @ Baseline	N = 6
Age /Years since Diagnosis	40.3 yrs / 12.5 yrs
Gender	4 (67%) Female
Race: African Caucasian Asian	2 (33%) 3 (50%) 1 (17%)
Bone Marrow Failure	3 (50%)
Thrombosis	3 (50%)
RBC Transfusion-dependent (prior 12 months)	5 (83%)
RBC Transfusions in prior 12 months, mean	13.7 units
Hb, mean g/dL* (range)	8.9 (7.5 – 10.2)
Reticulocyte count, mean $10^3/\mu L$ (range)	184 (125 – 235)
RBC C3 Opsonization, mean % (range)	15% (1% – 31%)
Total Bilirubin, mean mg/dL (range)	2.6 (0.8 – 5.3)
LDH x ULN (range)	0.9 (0.6 – 1.1)



Duration on ≥ 400mg BID:

Median 13 weeks

Range: 11 to 14 weeks

** Note, Hb data for one C5-INH-IR patient receiving transfusions was censored*

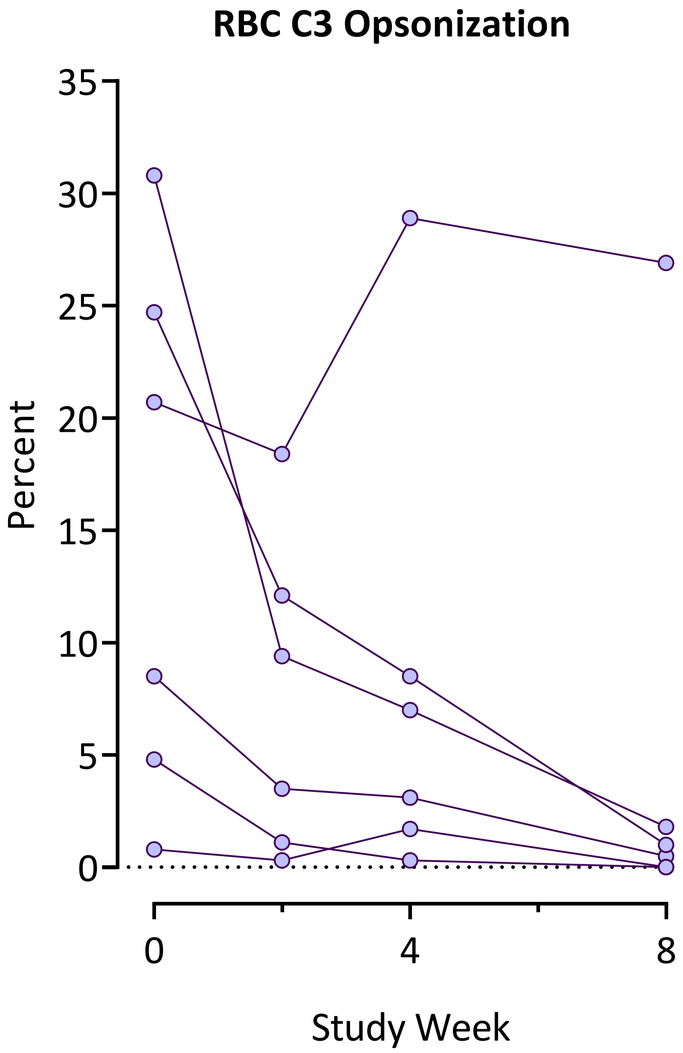
C5-inhibitor Inadequate Response Patients Experienced Significant Increase in Hemoglobin as a Result of Control of Hemolysis, with Reductions in C3 Opsonization, Bilirubin, and Reticulocytes

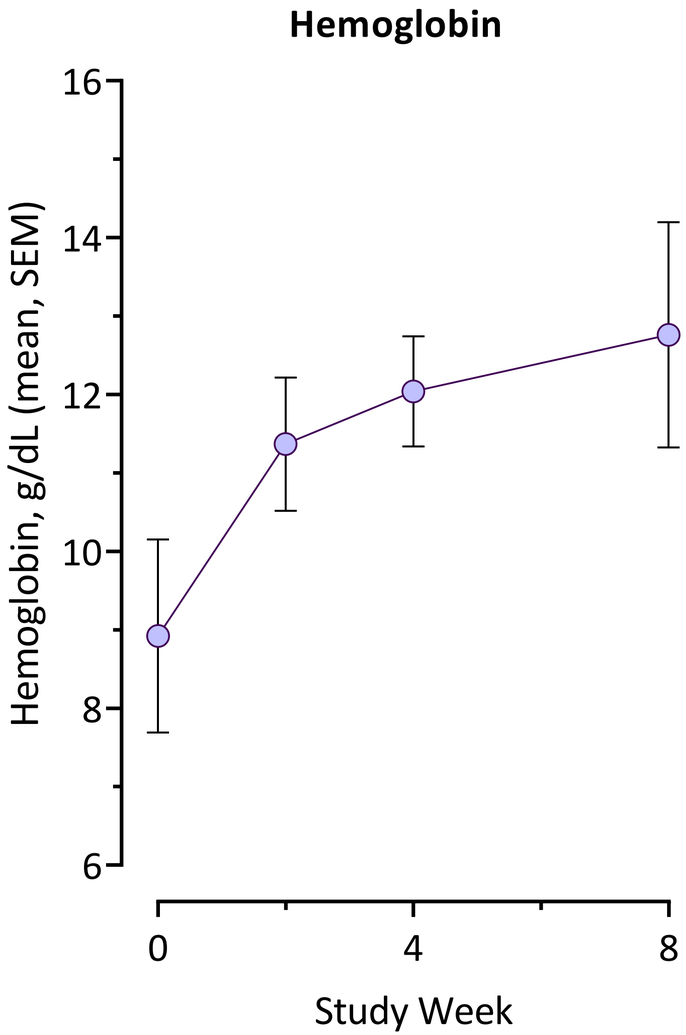
Response Parameter	Baseline N = 6	Last visit** N = 6	Change from Baseline
Hemoglobin g/dL, mean (SEM)*	8.9 (0.5)	12.2 (1.0)	+3.2 (0.6)
Hemoglobin > 12 g/dL, n (%)	0	3 (50%)	+3 (60%)
Hemoglobin > 10 g/dL, n (%)	2 (40%)	4 (80%)	+2 (40%)
RBC clone size %, mean (SEM)	48 (6)	77 (8)	+30 (7)
Reticulocytes 10^3/µL, mean (SEM)	184 (16)	139 (23)	-45 (18)
Patients with Reticulocytes ≤ 150,000/µL, n (%)	1 (17%)	4 (67%)	+3 (50%)
Total Bilirubin mg/dL, mean (SEM)	2.6 (0.7)	1.3 (0.3)	-1.3 (0.4)
RBC C3 Opsonization %, mean (SEM)	15 (4.9)	5 (4.4)	-10 (14)

** Note, Hb data for one C5-INH-IR patient receiving transfusions was censored*

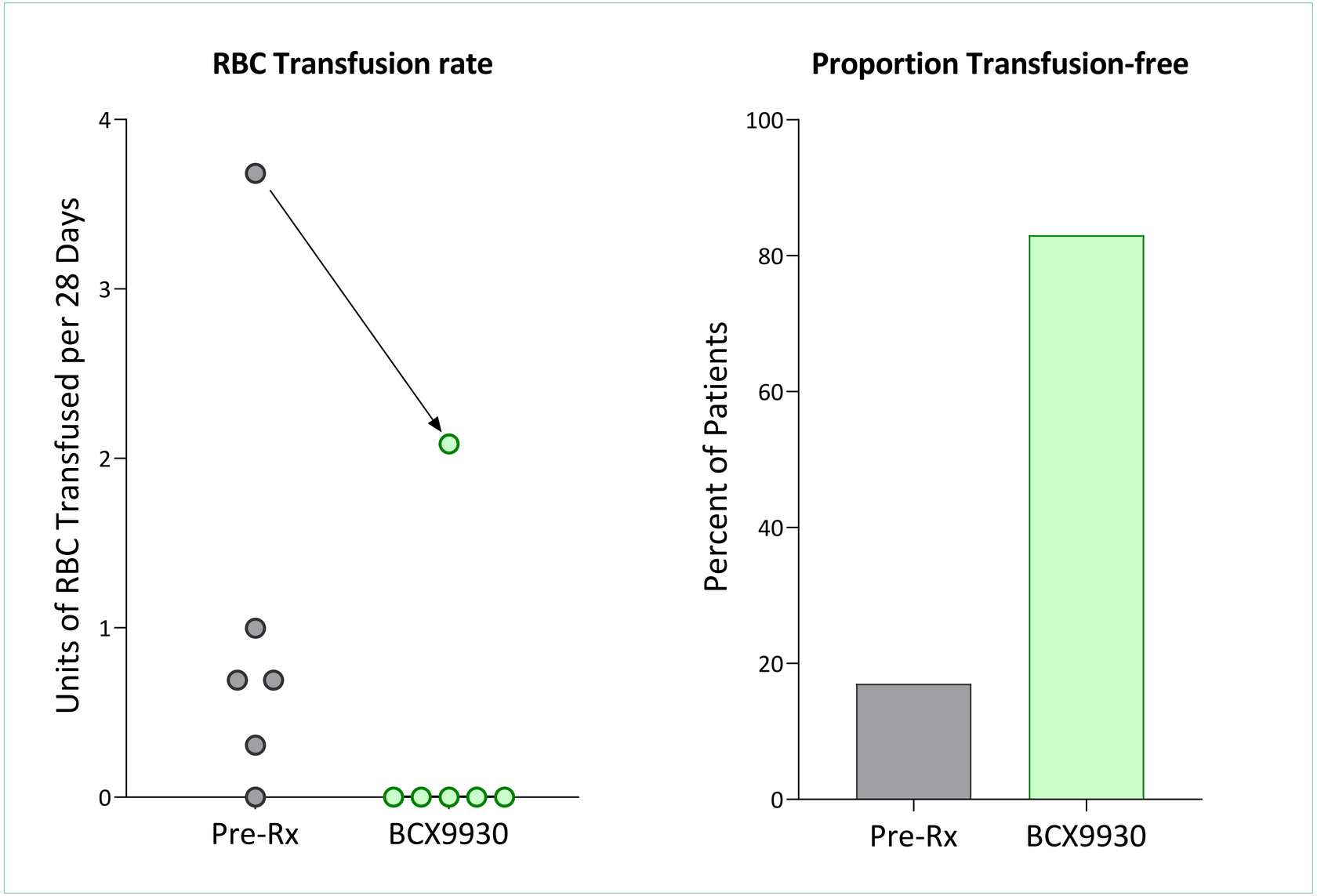
***Last treatment visit at 400mg or 500 mg BID*

RBC C3 Opsonization Showed a Pattern of Early Response in 5 of 6 Patients, and Hemoglobin also Responded Within Weeks



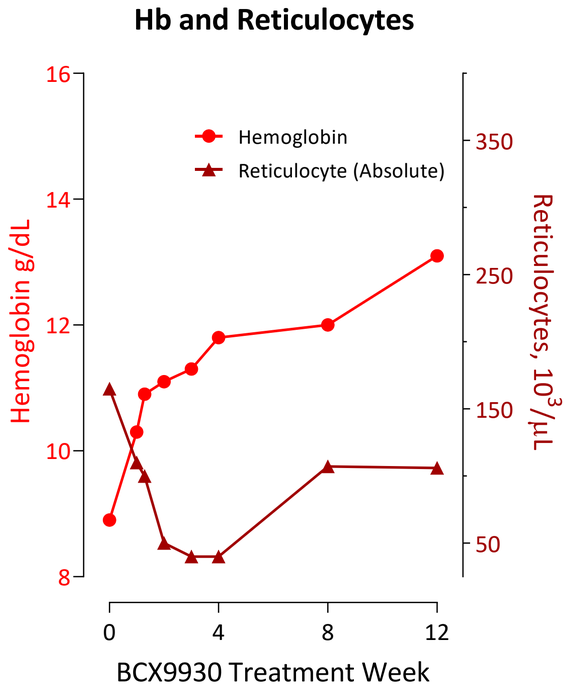


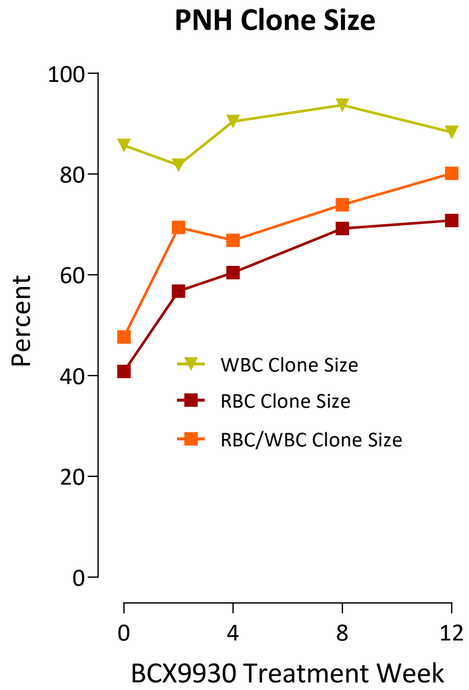
Transfusion Burden in C5-inhibitor Inadequate Response Subjects was Reduced to Zero in 5 of 6 Subjects – 83% of Patients were Free of Transfusions

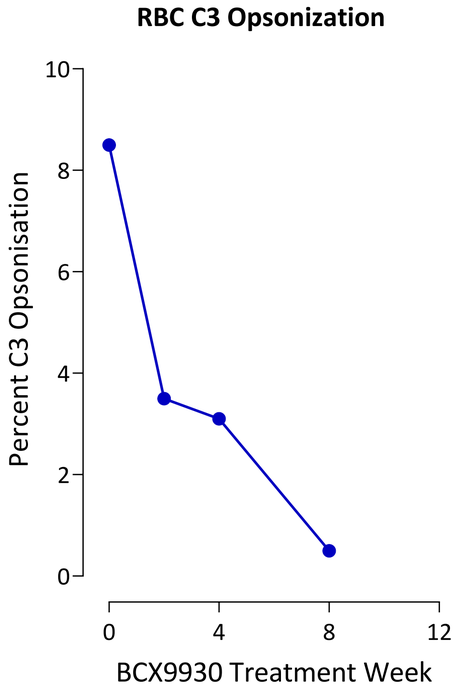


Example of C5-inhibitor Inadequate Responder Subject Response over the First 12 Weeks of Treatment with BCX9930

Patient N – 2 weeks Rx at 400 mg BID then 500 mg BID through week 12







Patient treated with eculizumab 1200 mg IV every two weeks for 3.8 years

- No RBC transfusions in 12 months before or on study
- Hb rose from 8.9 to 13.1 g/dL over 12 weeks
- Reticulocytes normalized, falling from 165 to 106 x 10^3/µL
- RBC/WBC clone size rose from 48% to 80%
- RBC C3 opsonization fell from 8.5% to 0.5%

Preliminary data

BCX9930 has been Safe and Well Tolerated in PNH Patients

Overall Safety

- No safety signals in routine monitoring of adverse events, vital signs, ECGs, or laboratory evaluations of hematology, clinical chemistry, coagulation, or urinalysis
- Treatment-emergent AEs of hemolysis occurred in 2/16, without changes to dosing
- No discontinuations or interruptions of dosing due to BCX9930-related treatment-emergent AEs

Related Adverse Events

- The most common BCX9930-related TEAE was mild-moderate headache in 8/10 C5-inhibitor naïve subjects and 2/6 C5-inhibitor inadequate response subjects, lasting 1-3 days, onset generally soon after commencing dosing – typical complement inhibition effect in PNH patients
- Mild or moderate drug rash observed in 6/16 subjects, onset generally in second week of dosing, lasting median of 10 days; all resolved without treatment interruption

BCX9930 Dose-ranging Proof-of-Concept Study in PNH: Conclusions

Overall outcomes seen in PNH patients on study at doses of 400 mg BID or 500 mg BID

- Hemoglobin increased, with relief of anemia
- Transfusions were avoided
- Intravascular and extravascular hemolysis were brought under control
- Consistent hematologic outcomes in both C5-inhibitor naïve and C5-inhibitor inadequate response patients

PK and PD profile

- Drug exposure and PD effects on complement activity in PNH patients were similar to that seen in healthy subjects

Dose-response and selection of dose for pivotal studies

- Optimized doses are in the range of 400-500 mg BID. Final selection of dose will be based on PK-PD modeling

Safety and tolerability

- BCX9930 dosing BID has been generally safe and well tolerated for up to over 50 weeks
- No safety signals have been seen

Conclusions and next steps

- These results support initiation of pivotal trials in PNH
- Pivotal trials in PNH are planned for start in 2H 2021

BCX9930 Program Advancing Rapidly

- BioCryst reaches agreement with FDA that change from baseline in hemoglobin is the primary endpoint for pivotal PNH trials

- Pivotal trials (500 mg bid) in PNH to begin in 2H 2021
  - PNH patients not being treated with a C5 inhibitor
  - PNH patients with an inadequate response to C5 inhibitor therapy

- Proof-of-concept trial(s) (500 mg bid) in renal complement-mediated diseases to begin 2H 2021

Goal for BCX9930 in PNH: Broad indication as oral monotherapy for all PNH patients

Fibrodysplasia Ossificans Progressiva (FOP)

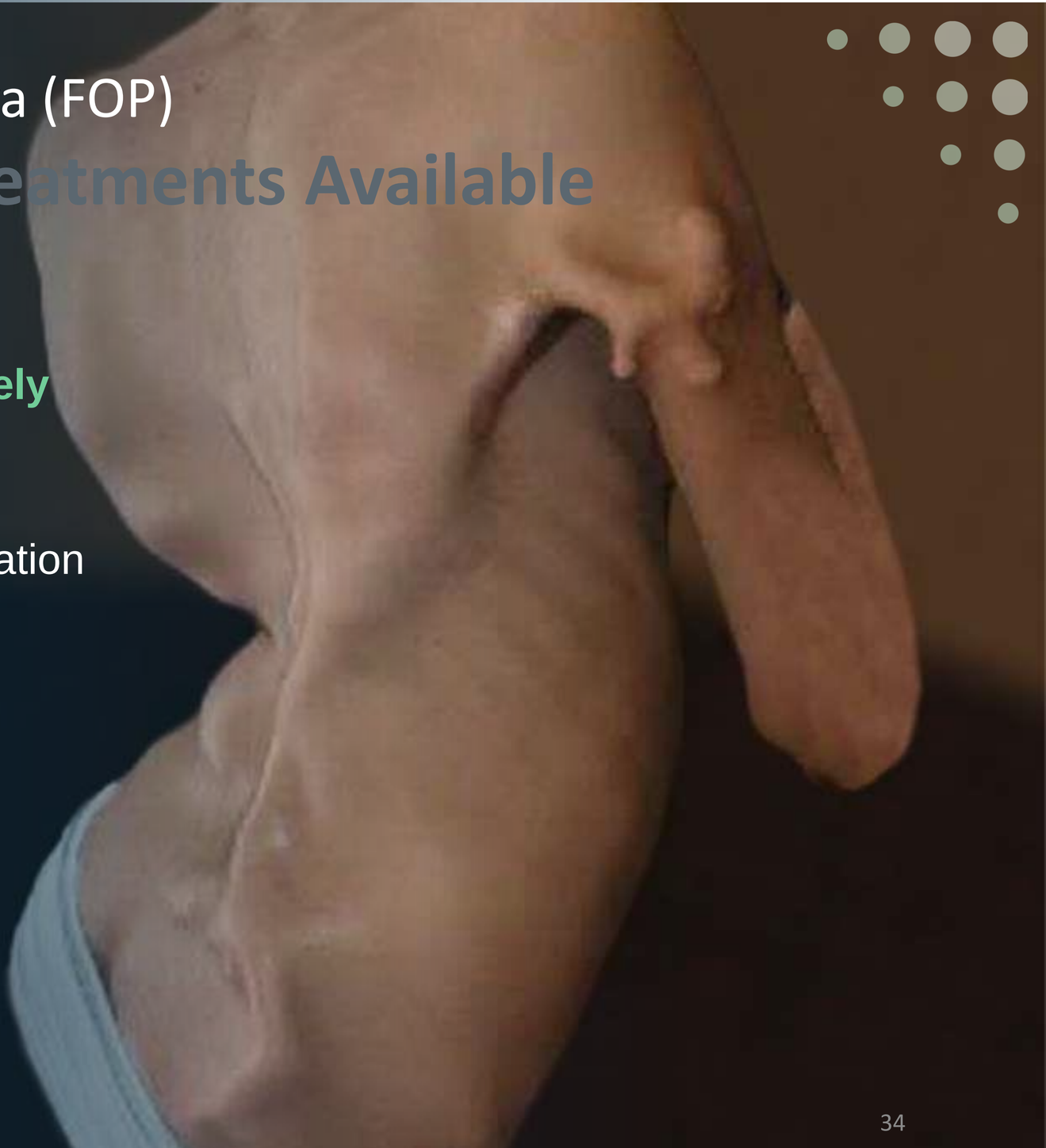
Devastating Disease; No Treatments Available

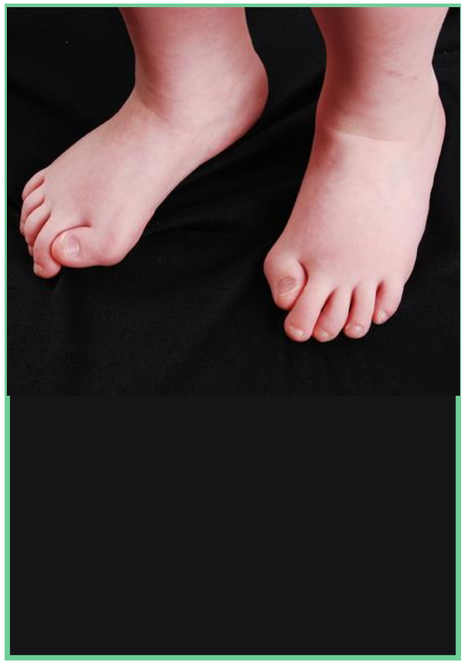
Rare disease that **affects approximately 1 in 2 million people** worldwide

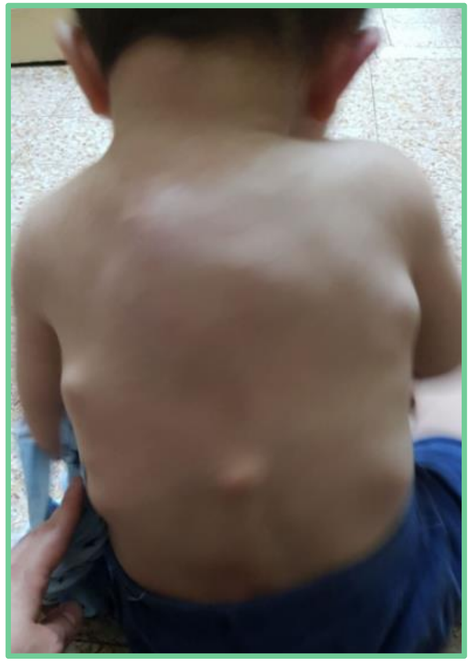
Irregular formation of bone or ossification in muscles, tendons or soft tissue

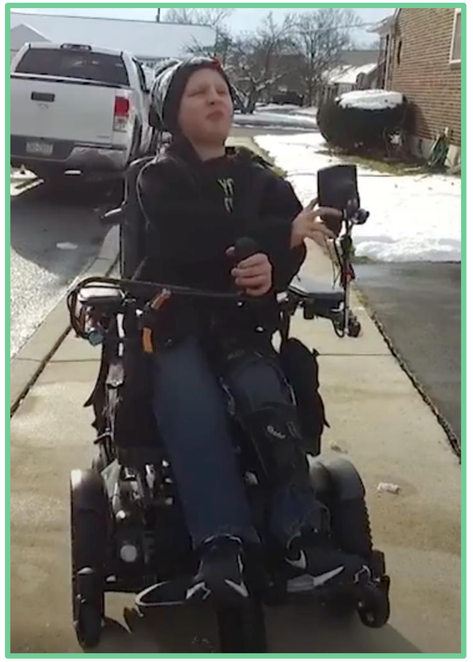
Currently no approved treatments for FOP

Results in loss of function, deformities and **a severely disabling condition**









ifopa.org

BCX9250 Phase 1 Healthy Subject Trial Design

Randomized, double-blind, placebo-controlled, dose-ranging trial in healthy volunteers

Objective: to evaluate safety, tolerability, and pharmacokinetics of single ascending doses (SAD) and multiple ascending doses (MAD) of orally administered BCX9250

Part 1

Single ascending dose

- 8 subjects per cohort
  - 6 active, 2 placebo

Dose levels evaluated:

- 5mg
- 10mg
- 15mg (fed and fasted)
- 25mg

Part 2

Multiple ascending dose, once daily (QD) for 7 days

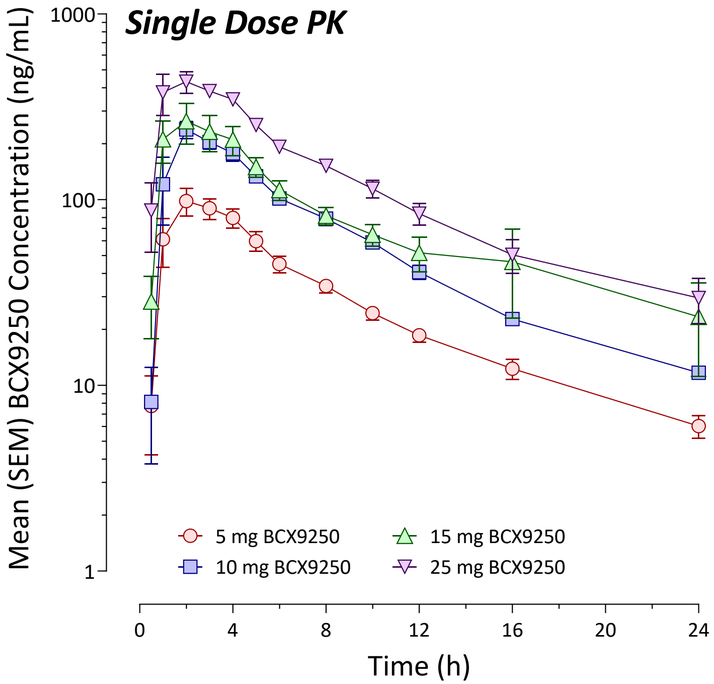
- 12 subjects per cohort
  - 10 active, 2 placebo

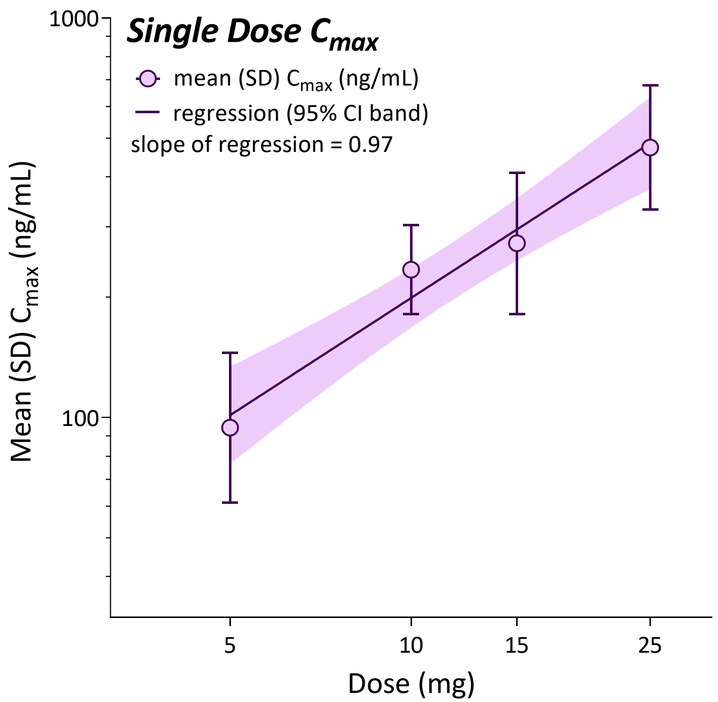
Dose levels evaluated:

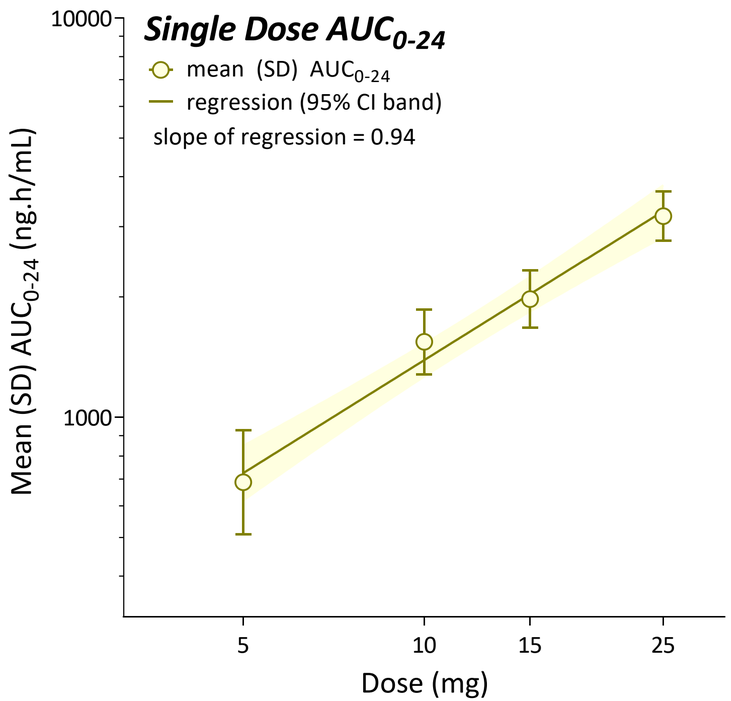
- 5mg
- 10mg
- 15mg
- 20mg

BCX9250 SAD PK Profile and Dose-exposure Analysis

BCX9250 exposure was approximately linear and dose proportional over the doses evaluated

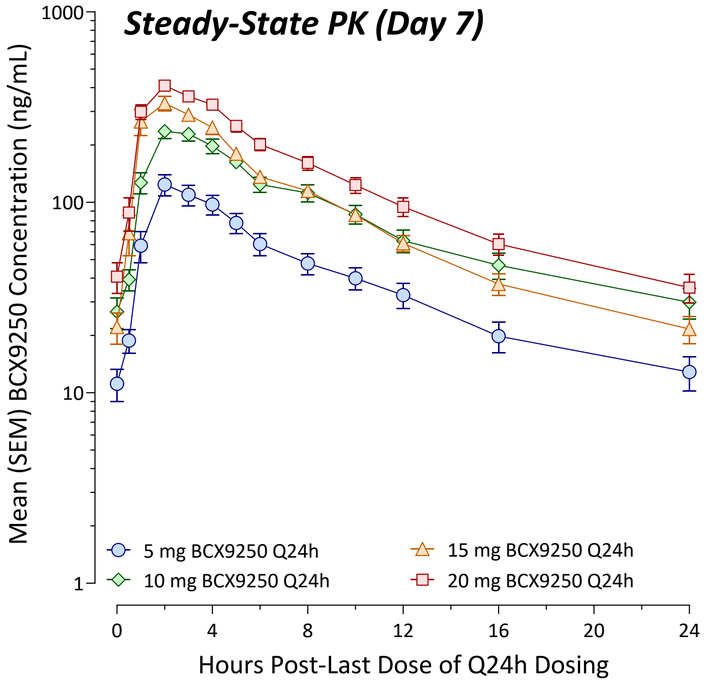


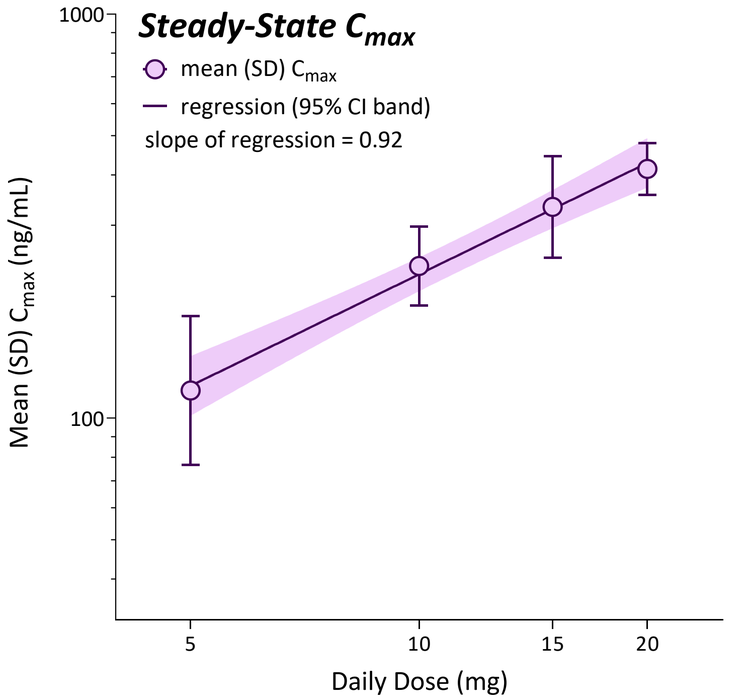


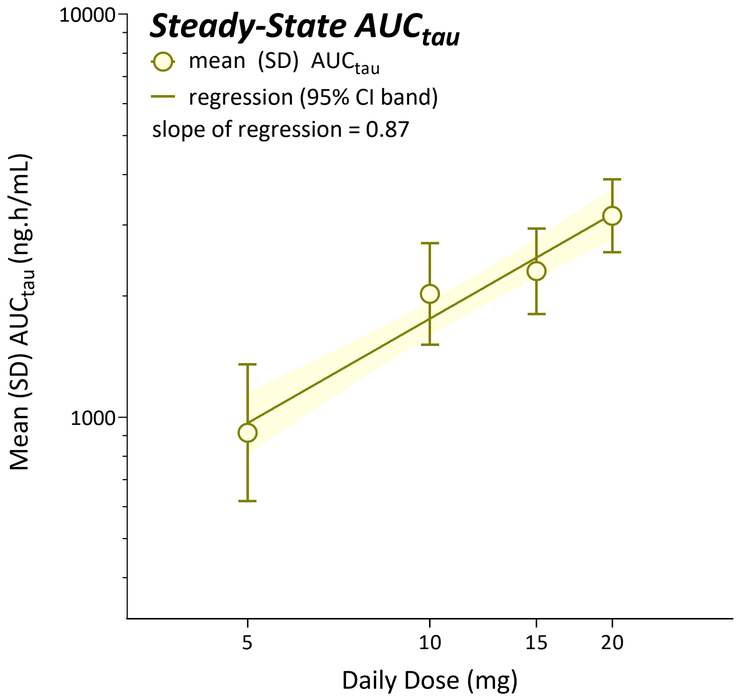


BCX9250 MAD PK Profile and Dose-exposure Analysis

BCX9250 steady-state exposure was approximately linear and dose proportional over the doses evaluated, with minimal accumulation relative to the first dose







BCX9250 Phase 1 Trial: Summary of Adverse Events

Category of Treatment-Emergent Adverse Event (TEAE)	Single Ascending Doses (SAD)						Multiple Ascending Doses (MAD)				
All data is reported as subject incidence, n (%)	**Placebo**	**BCX9250**					**Placebo**	**BCX9250**			
	(n=8)	**5 mg (n=6)**	**10 mg (n=6)**	**15 mg Fasted (n=6)[a]**	**15 mg Fed (n=6)**	**25 mg (n=6)**	**(n=7)[b]**	**5 mg (n=10)**	**10 mg (n=10)**	**15 mg (n=10)**	**20 mg (n=10)**
At least one TEAE	4 (50.0)	0	0	4 (66.7)	3 (50.0)	0	5 (71.4)	6 (60.0)	3 (30.0)	6 (60.0)	6 (60.0)
Drug-related TEAEs	3 (37.5)	0	0	2 (33.3)	0	0	4 (57.1)	0	3 (30.0)	1 (10.0)	0
Grade 3 or 4 TEAEs	0	0	0	0	0	0	0	0	0	0	0
Serious TEAE	0	0	0	0	0	0	0	0	0	0	0
Drug-related serious TEAE	0	0	0	0	0	0	0	0	0	0	0
TEAE leading to study discontinuation	0	0	0	0	0	0	0	0	0	0	0
Drug-related TEAE leading to study discontinuation	0	0	0	0	0	0	0	0	0	0	0
TEAEs reported by 2 or more subjects [c]											
Medical device site reaction [d]	0	0	0	2 (33.3)	1 (16.7)	0	0	2 (20.0)	0	1 (10.0)	3 (30.0)
Headache	2 (25.0)	0	0	1 (16.7)	0	0	1 (14.3)	0	2 (20.0)	2 (20.0)	0
Vessel puncture site pain	1 (12.5)	0	0	0	0	0	1 (14.3)	1 (10.0)	0	0	2 (20.0)
Abdominal discomfort	2 (25.0)	0	0	0	0	0	0	0	1 (10.0)	0	0
Abdominal pain	1 (12.5)	0	0	0	0	0	0	0	1 (10.0)	0	1 (10.0)
Diarrhea	1 (12.5)	0	0	0	0	0	0	0	2 (20.0)	0	0
Constipation	0	0	0	0	0	0	1 (14.3)	0	0	1 (10.0)	0
Flatulence	0	0	0	0	0	0	1 (14.3)	0	1 (10.0)	0	0
Nausea	1 (12.5)	0	0	1 (16.7)	0	0	0	0	0	0	0
Cough	1 (12.5)	0	0	0	0	0	0	0	1 (10.0)	0	0

[a] One subject discontinued from study after completing first dose (fasted) and was replaced for the second dose (fed).

[b] Only one placebo subject was enrolled in MAD 20 mg cohort. The last subject was not enrolled due to impact of COVID-19 on screening.

[c] All TEAEs were mild except for one event of moderate myalgia in the MAD 10 mg dose group, not related to study drug.

[d] Reported event: electrode site (skin) irritation due to ECG lead placement

Cash position (in millions)

Cash, cash equivalents, restricted cash & investments at December 31, 2020	$303
Cash, cash equivalents, restricted cash & investments at March 31, 2021	$244
Senior credit facility [A]	$125

A – From Athyrium Capital Management, $125M interest-only for 5-year term

In the launch period for ORLADEYO, the company is not providing specific revenue or operating expense guidance. Based on our expectations for revenue, operating expenses, and our option to access an additional $75 million from our existing credit facility, we believe our current cash runway takes us into 2023.

May 2021
Corporate Presentation