



2022 J.P. MORGAN HEALTHCARE CONFERENCE

Jon Stonehouse | President and Chief Executive Officer

FORWARD-LOOKING STATEMENTS

BioCryst's presentation may contain forward-looking statements, including statements regarding future results, unaudited and forward-looking financial information and company performance or achievements. These statements are subject to known and unknown risks and uncertainties which may cause our actual results, performance or achievements to be materially different from any future results or performances expressed or implied in this presentation.

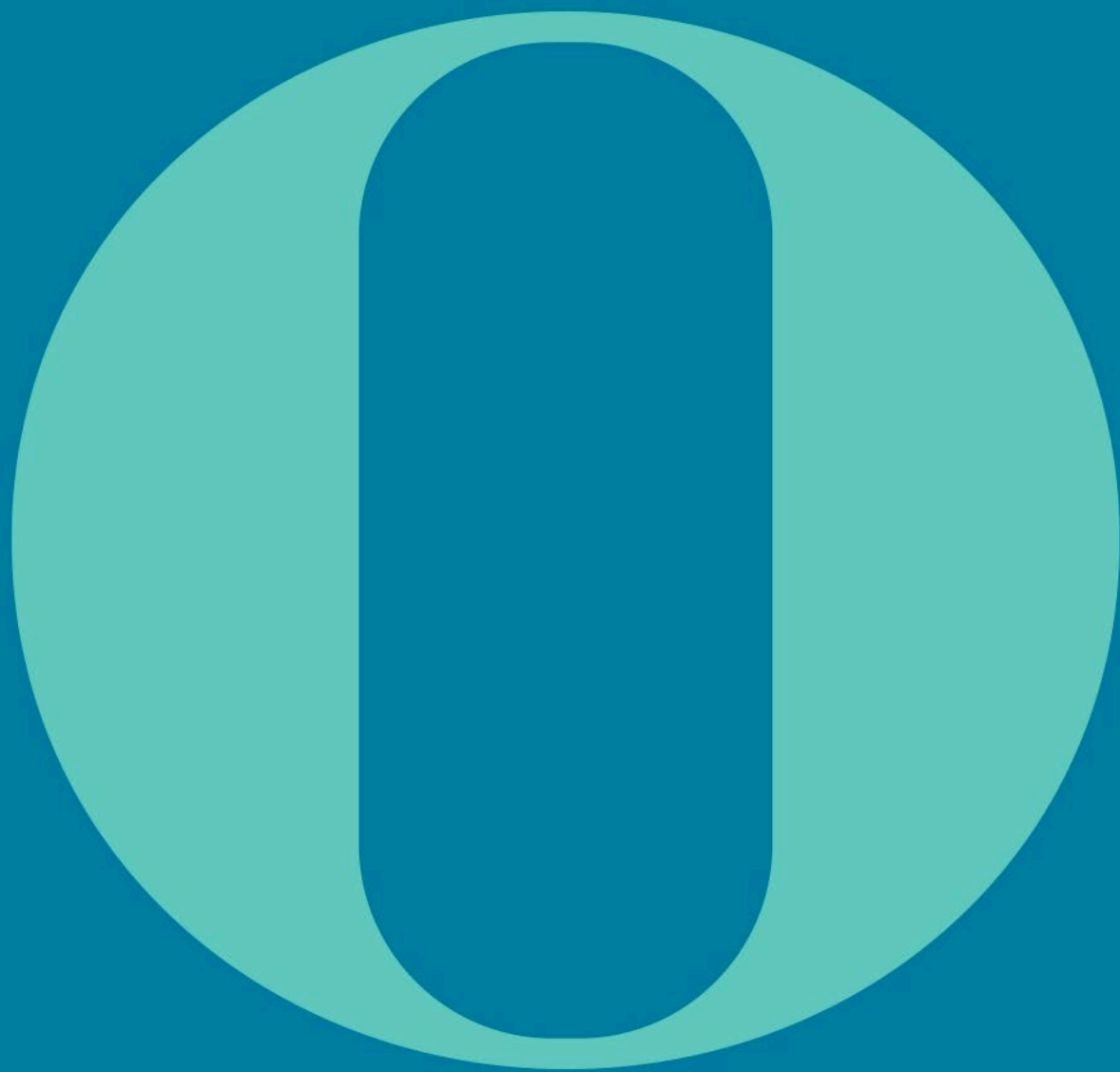
You should not place undue reliance on the forward-looking statements. For additional information, including important risk factors, please refer to BioCryst's documents filed with the SEC and located at ir.biocryst.com/financial-information/sec-filings



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Orladeyo[®]

(berotralstat) capsules 150 mg



\$122M

\$250M



This is Kissa's experience with ORLADEYO® (berotralstat). Individual results may vary.

**Please see Important Safety Information and review the Prescribing Information,
including the Patient Information, at [ORLADEYO.com](https://www.OracleBio.com)**

ORLADEYO® (berotralstat) PROVIDES SUSTAINED ATTACK RATE REDUCTION¹

Patients who completed 96 weeks of treatment saw sustained reductions in their HAE attack rates, demonstrating the durability of ORLADEYO

21 patients who were randomized to ORLADEYO 150 mg at the beginning of APeX-2 and completed 96 weeks of treatment demonstrated a decline in mean attack rate per 4 weeks from baseline to 96 weeks of treatment^b

SEM, standard error of mean.

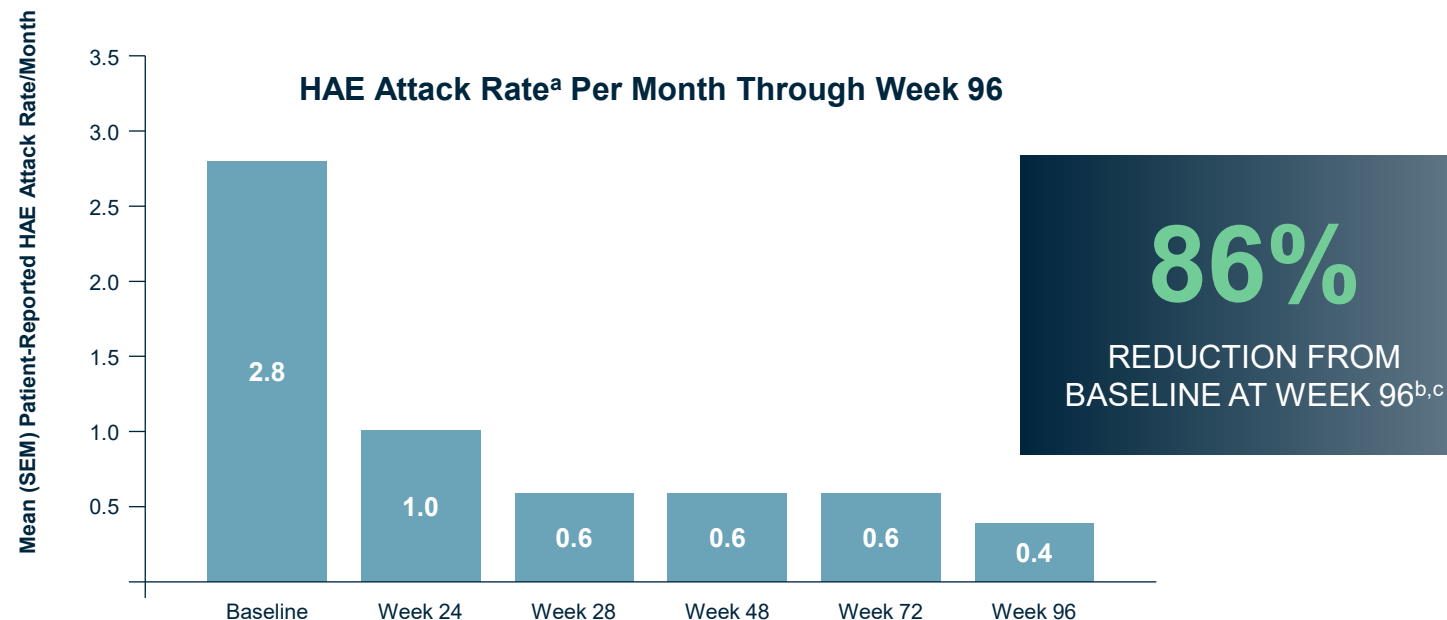
^aDue to study design, investigator-confirmed attack rates were reported only during the first 48 weeks, while patient-reported attack rates were reported during weeks 49 to 96. For consistency across the entire 96 weeks, only patient-reported attack rates are reported. For analysis purposes, 1 month was defined as 4 weeks of treatment.¹

^bThis reflects an ad hoc analysis of interim data.^{1,2}

^c86% attack rate reduction from baseline to week 96 was seen for patients who completed 96 weeks of treatment with ORLADEYO 150 mg (n = 21).¹

1. Kiani S, et al. Presented at: European Academy of Allergy and Clinical Immunology Hybrid Congress; July 10-12, 2021.

2. Data on file, BioCryst Pharmaceuticals, Inc.



IN 16 OF THE LAST 17 MONTHS OF TREATMENT, MEDIAN ATTACK RATE WAS 0 ATTACKS PER MONTH^b

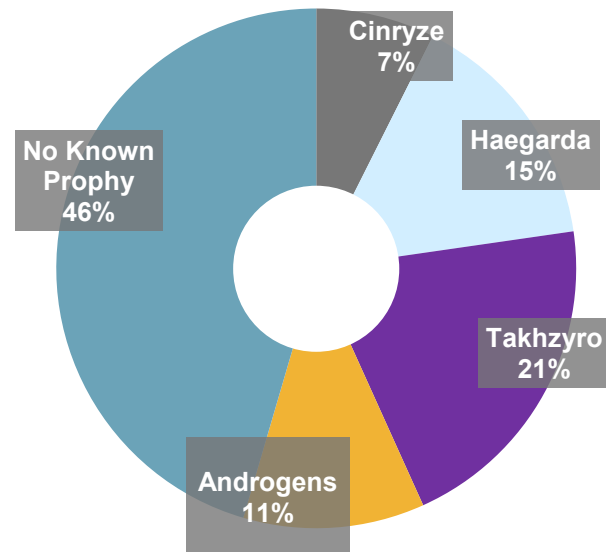
SELECT IMPORTANT SAFETY INFORMATION

The most common adverse reactions (≥10% and higher than placebo) in patients receiving ORLADEYO were abdominal pain, vomiting, diarrhea, back pain, and gastroesophageal reflux disease.

Please see Important Safety Information.

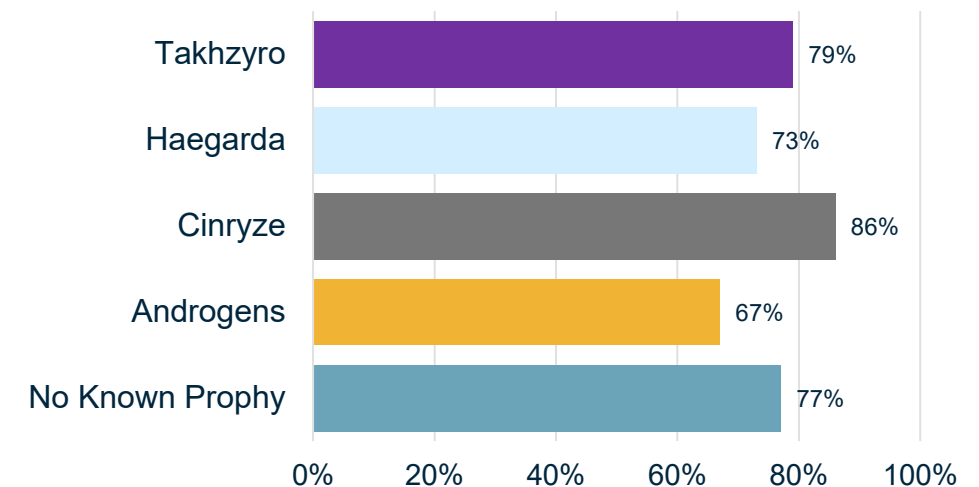
PATIENTS ON ORLADEYO: COMPARISON BY PREVIOUS HAE MEDICATION

Previous HAE Medication



Source: Estimates from internal analysis of ORLADEYO patients starting therapy Dec'20-Nov'21, consented with medical history

% of Patients Staying on ORLADEYO >6 months

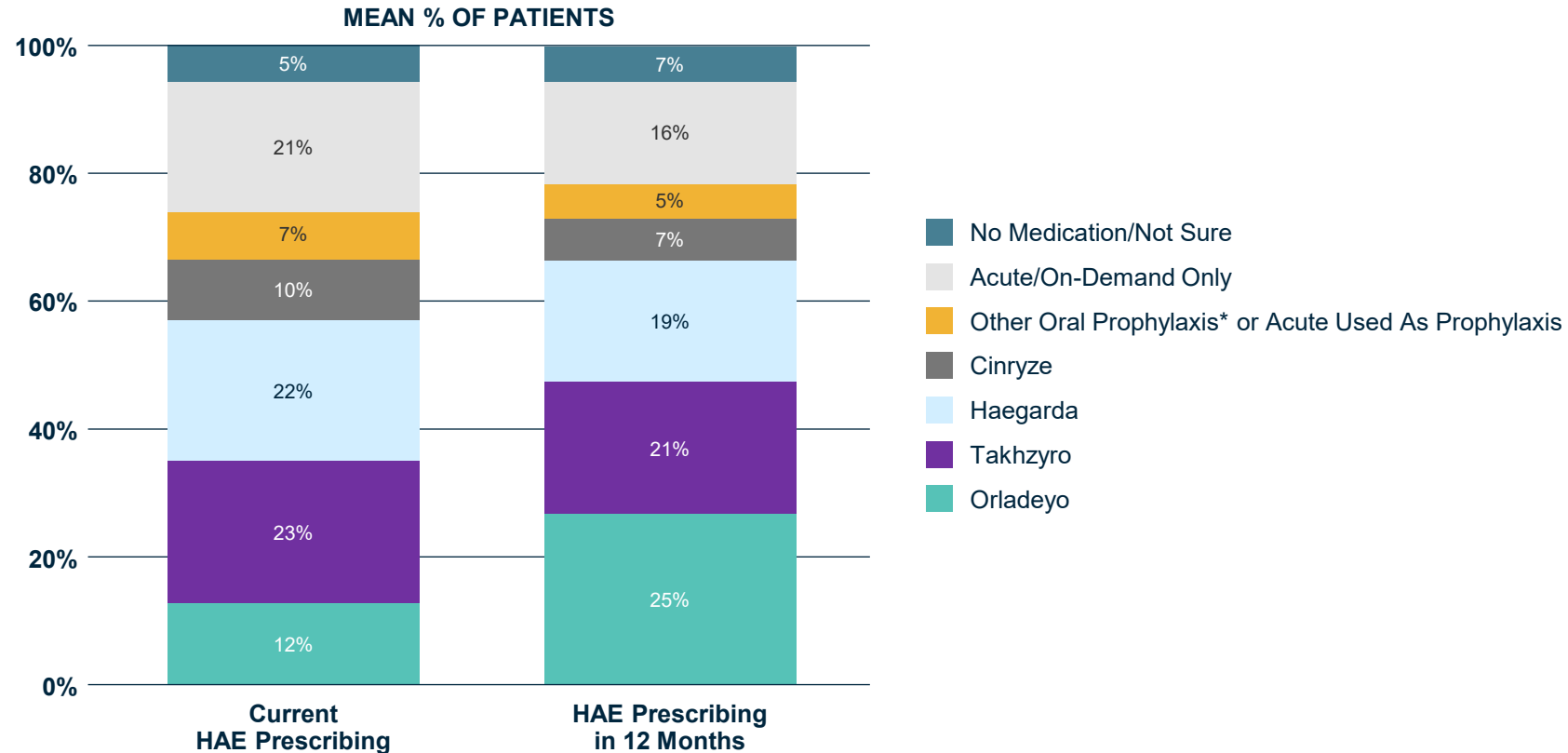


Source: Based on non-clinical Paid/PAP patients starting on therapy on or before June 10, 2021

A RECENT SURVEY OF ALLERGISTS/IMMUNOLOGISTS, TREATING ON AVERAGE SEVEN HAE PATIENTS EACH, SUGGESTS THEY EXPECT USE OF **ORLADEYO** TO DOUBLE OVER THE NEXT 12 MONTHS TO BE THEIR MOST PRESCRIBED PROPHYLAXIS

Future Prescribing of HAE Medications for **Prophylaxis** (Current & In Next 12 Months)

All Qualified Respondents (n=60)



*(e.g., androgens, tranexamic acid)

Source: BioCryst Proprietary Market Research conducted with 60 Allergist/Immunologists in August 2021

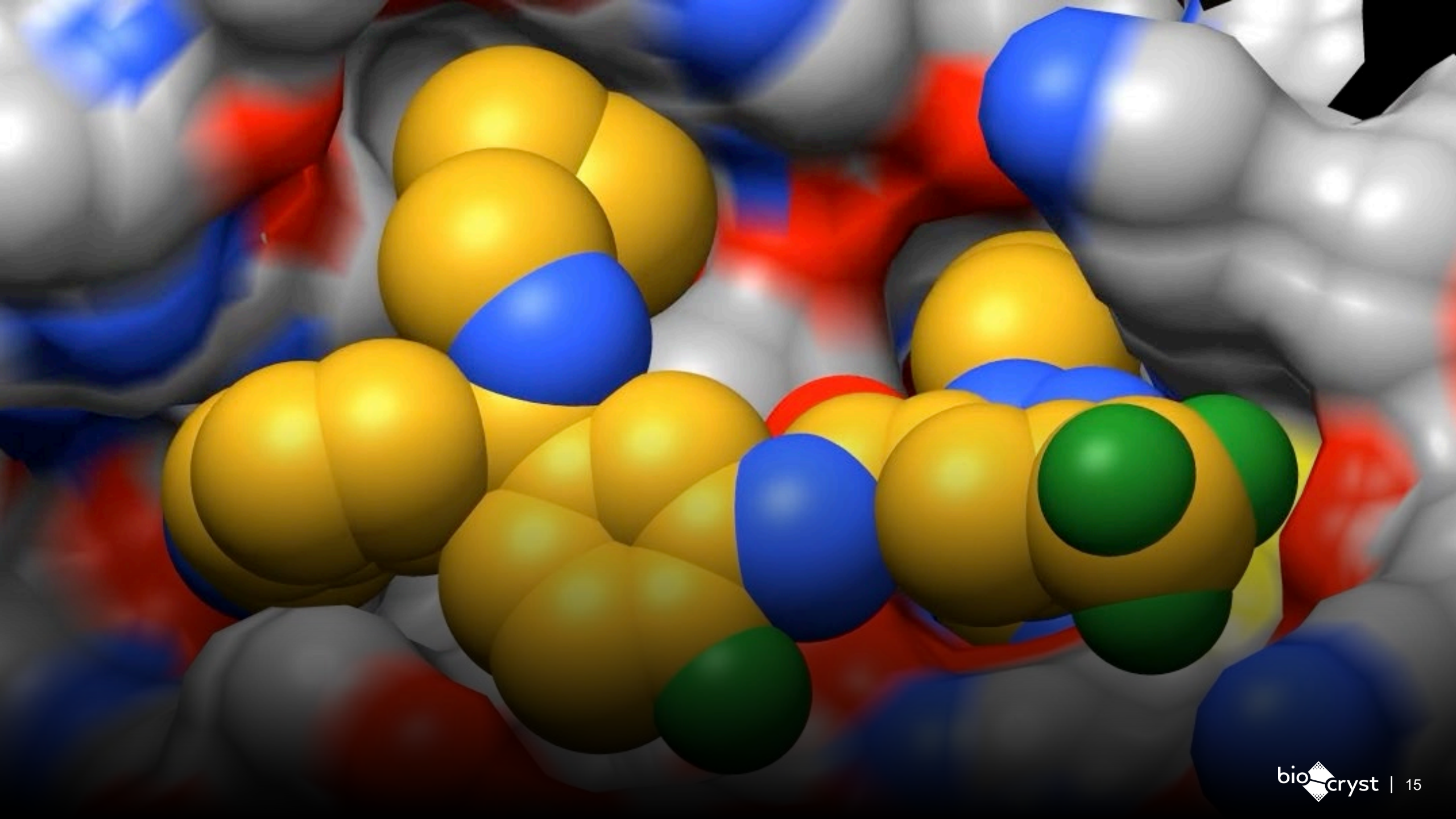
\$250M

\$1B



OUR DRUG DISCOVERY PLATFORM







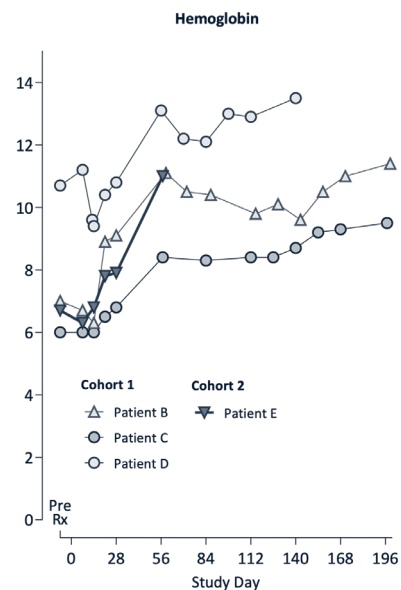
OUR DRUG DISCOVERY PLATFORM





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1 YEAR AGO: PROOF OF CONCEPT IN PATIENTS WITH PNH



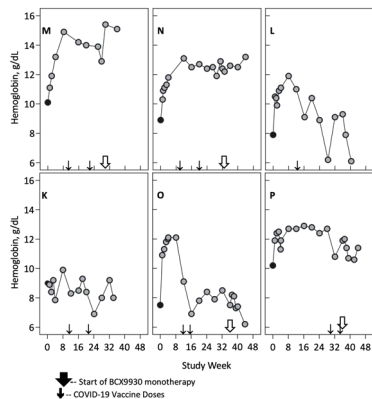
- Mean increase in Hb from baseline of 3.8 g/dL
- Hb maintained at 400 mg BID without RBC transfusions
- Mean RBC PNH clone size relative to granulocyte clone size increased to 94% from 48% pre-Rx



Patient	Duration at 400 mg BID	Hemoglobin g/dL		RBC Clone Size % of Granulocyte Clone Size		# of Transfusions @ 200/400 mg
		Pre-Rx	Most Recent	Pre-Rx	Most Recent	
B	56 days	7.0	11.4	42%	100%	0
C	57 days	6.0	9.5	53%	97%	0
D	56 days	10.7	13.5	60%	87%	0
E	43 days	6.7	11.0	36%	92%	0
Mean	53 days	7.6	11.4	48%	94%	0

TODAY: CLINICAL BENEFIT WITH BCX9930

Hemoglobin by Patient, BCX9930 POC Study

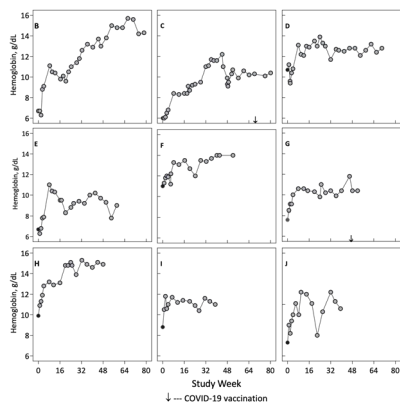


Endpoints as Specified in REDEEM-1	POC Data [§]	
	All Patients	Excluding "K"
Mean (SEM) CFB in Hb (g/dL), weeks 12, 16, 20, 24	+2.3 (0.9)*	+2.7 (0.8)
Proportion RBC transfusion-free, day 14 to week 24	67%	80%
Mean (SEM) number of units packed RBC transfused, day 14 to week 24	2.8 (1.9)	1.2 (1.2)
Mean (SEM) CFB in FACIT-fatigue scale score (units), weeks 12, 16, 20, 24	+3.9 (4.3)	+3.4 (5.2)

[§]CFB in Hb was set to 0 for patient K, who remained transfusion-dependent

REDEEM-1 is powered at 90% to detect a difference in mean change from baseline of hemoglobin of 2 g/dL

Hemoglobin by Patient, BCX9930 POC Study

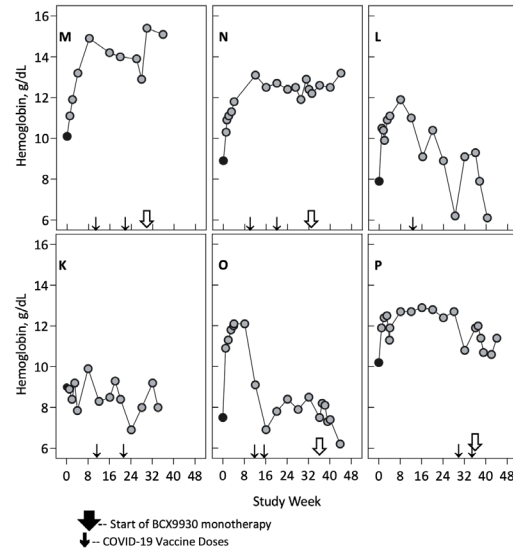


Endpoints as Specified in REDEEM-2	POC Data for Dosing Period at 400 mg or 500 mg BID [§]
Mean (SEM) CFB in Hb (g/dL), week 12	+3.7 (0.5)
Proportion RBC transfusion-free, day 14 to week 12	100%
Mean (SEM) number of units packed RBC transfused, day 14 to week 12	0
Mean (SEM) Percent CFB in LDH (U/L), week 12	-65% (7%)
Mean (SEM) CFB in FACIT-fatigue scale score (units), week 12	+7.1 (2.3)

REDEEM-2 is powered at 90% to detect a difference in mean change from baseline of hemoglobin of 2.15 g/dL



Hemoglobin by Patient, BCX9930 POC Study



Endpoints as Specified in REDEEM-1

Mean (SEM) CFB in Hb (g/dL), weeks 12, 16, 20, 24

Proportion RBC transfusion-free, day 14 to week 24

Mean (SEM) number of units packed RBC transfused, day 14 to week 24

Mean (SEM) CFB in FACIT-fatigue scale score (units), weeks 12, 16, 20, 24

*CFB in Hb was set to 0 for patient K, who remained transfusion-dependent

POC Data[§]

All Patients

Excluding "K"

+2.3 (0.9)*

+2.7 (0.8)

67%

80%

2.8 (1.9)

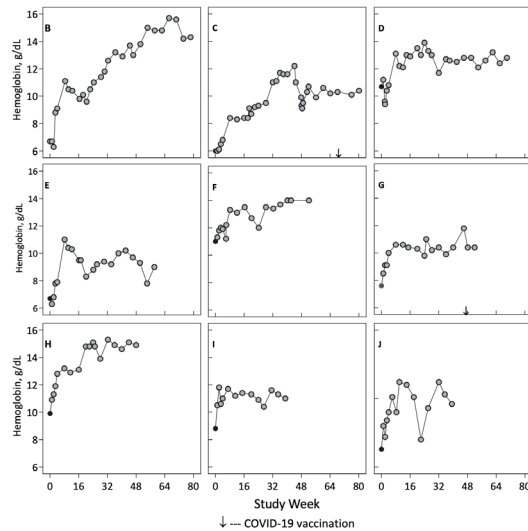
1.2 (1.2)

+3.9 (4.3)

+3.4 (5.2)

REDEEM-1 is powered at 90% to detect a difference in mean change from baseline of hemoglobin of 2 g/dL

Hemoglobin by Patient, BCX9930 POC Study



Endpoints as Specified in REDEEM-2

Mean (SEM) CFB in Hb (g/dL), week 12

Proportion RBC transfusion-free, day 14 to week 12

Mean (SEM) number of units packed RBC transfused, day 14 to week 12

Mean (SEM) Percent CFB in LDH (U/L), week 12

Mean (SEM) CFB in FACIT-fatigue scale score (units), week 12

POC Data for Dosing Period at 400 mg or 500 mg BID[§]

+3.7 (0.5)

100%

0

-65% (7%)

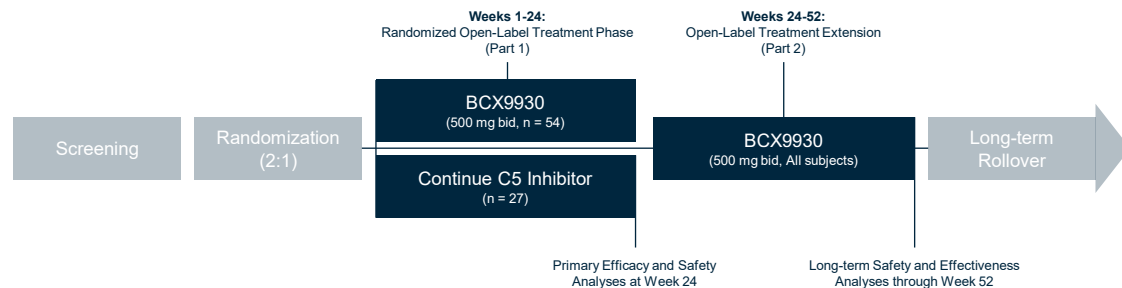
+7.1 (2.3)

REDEEM-2 is powered at 90% to detect a difference in mean change from baseline of hemoglobin of 2.15 g/dL

PIVOTAL TRIALS IN PNH: NOW ENROLLING



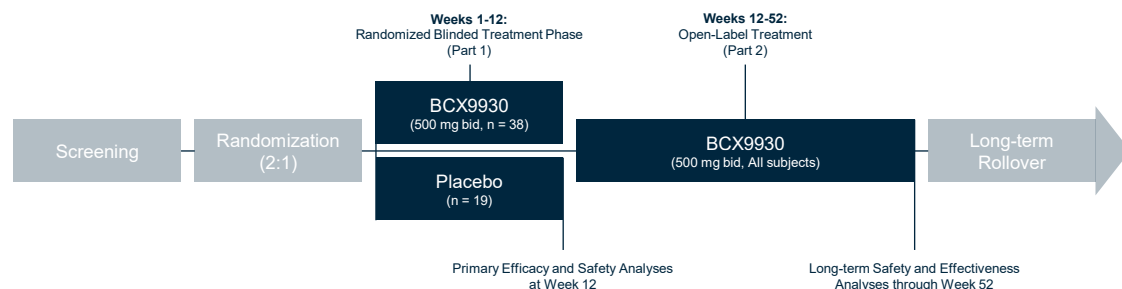
Pivotal Trial of BCX9930 as Oral Monotherapy in PNH Patients with Inadequate Response to C5-Inhibitor Therapy



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

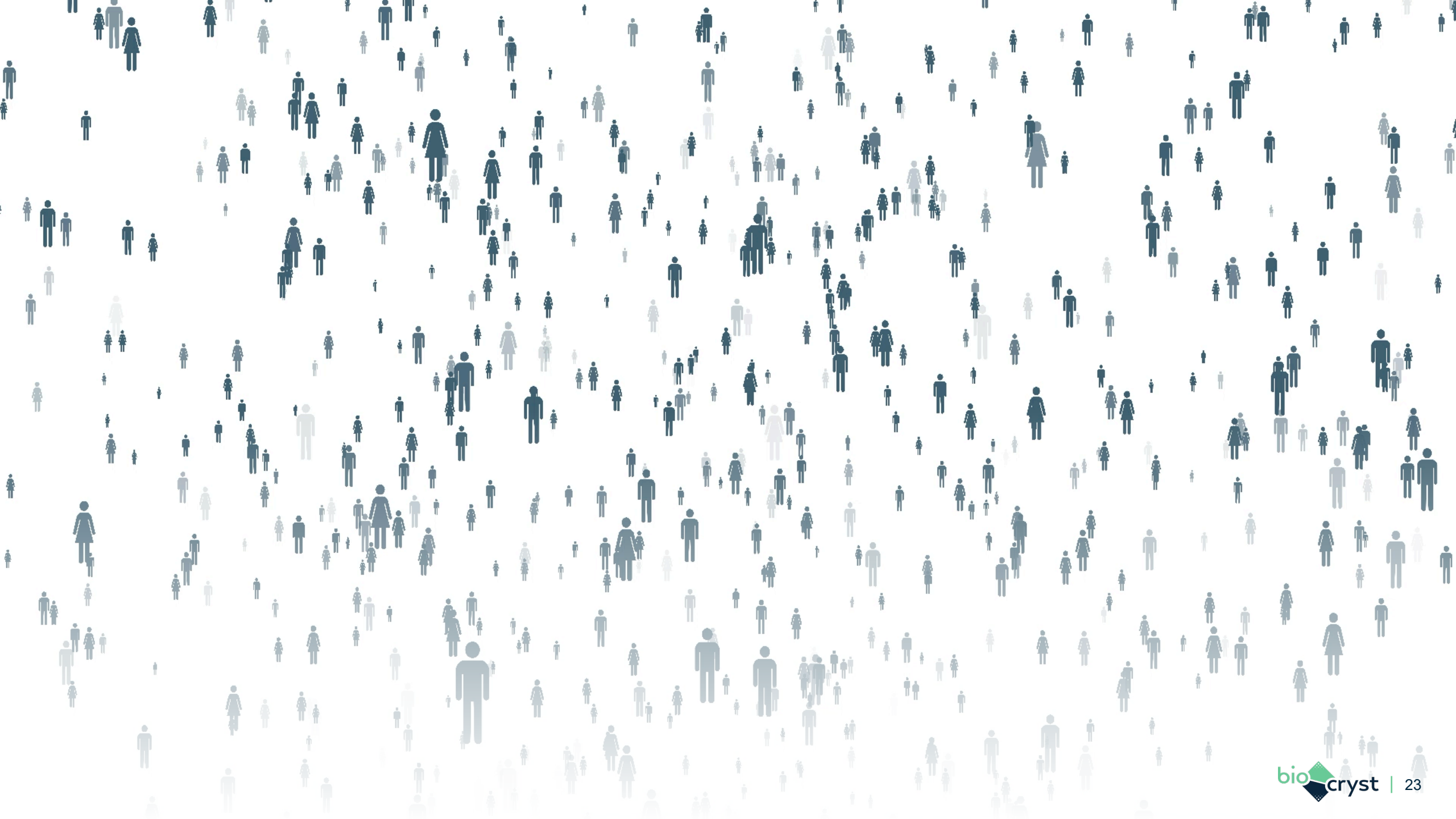


Pivotal Trial of BCX9930 as Oral Monotherapy in PNH Patients Not Currently Receiving C5 Inhibitor Therapy



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





“

*WHAT I'D LIKE TO SEE IN THE FUTURE IS SOMETHING
I COULD DO DAILY*

”

“

*I'D RATHER TAKE A PILL DAILY THAN GO EVERY EIGHT
WEEKS TO GET AN INFUSION*

”

“

I WISH IT WAS A LITTLE EASIER... BUT IT'S NOT

”

“

*HONESTLY, THAT'S EXACTLY THE KIND OF THING I'VE
BEEN LOOKING FOR...SOMETHING LIKE THIS*

”

“

*MY PIE IN THE SKY DREAM SCENARIO IS TAKING A PILL
TWICE A DAY RATHER THAN GOING AND GETTING ANY
KIND OF STICKS AND INFUSIONS*

”

“

*I JUST TAKE SOMETHING ORALLY? TAKE A PILL EVERY
DAY? I'D BE IN SO QUICK.*

”

EXPANDING INTO MORE INDICATIONS WITH BCX9930

IgAN



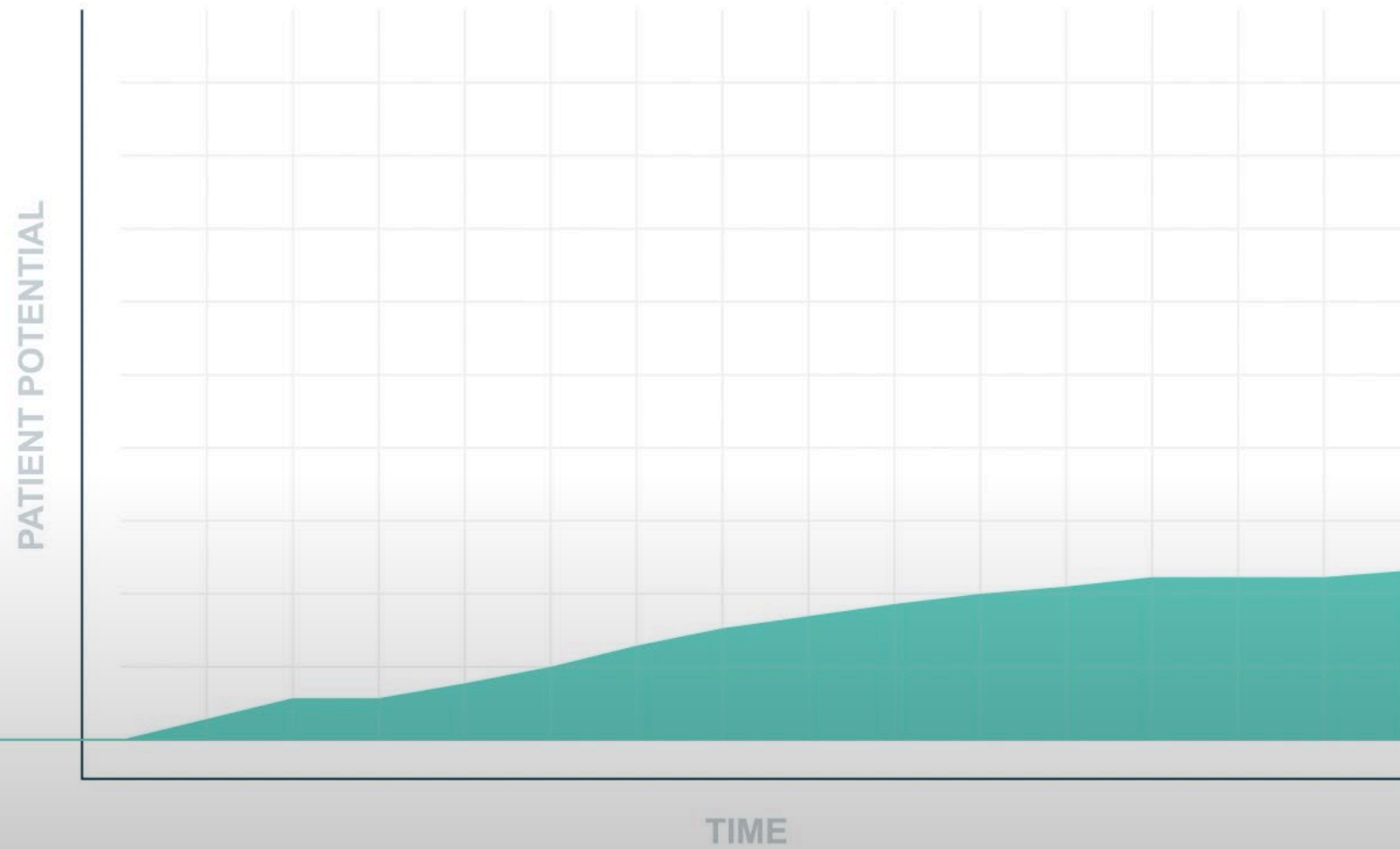
C3G



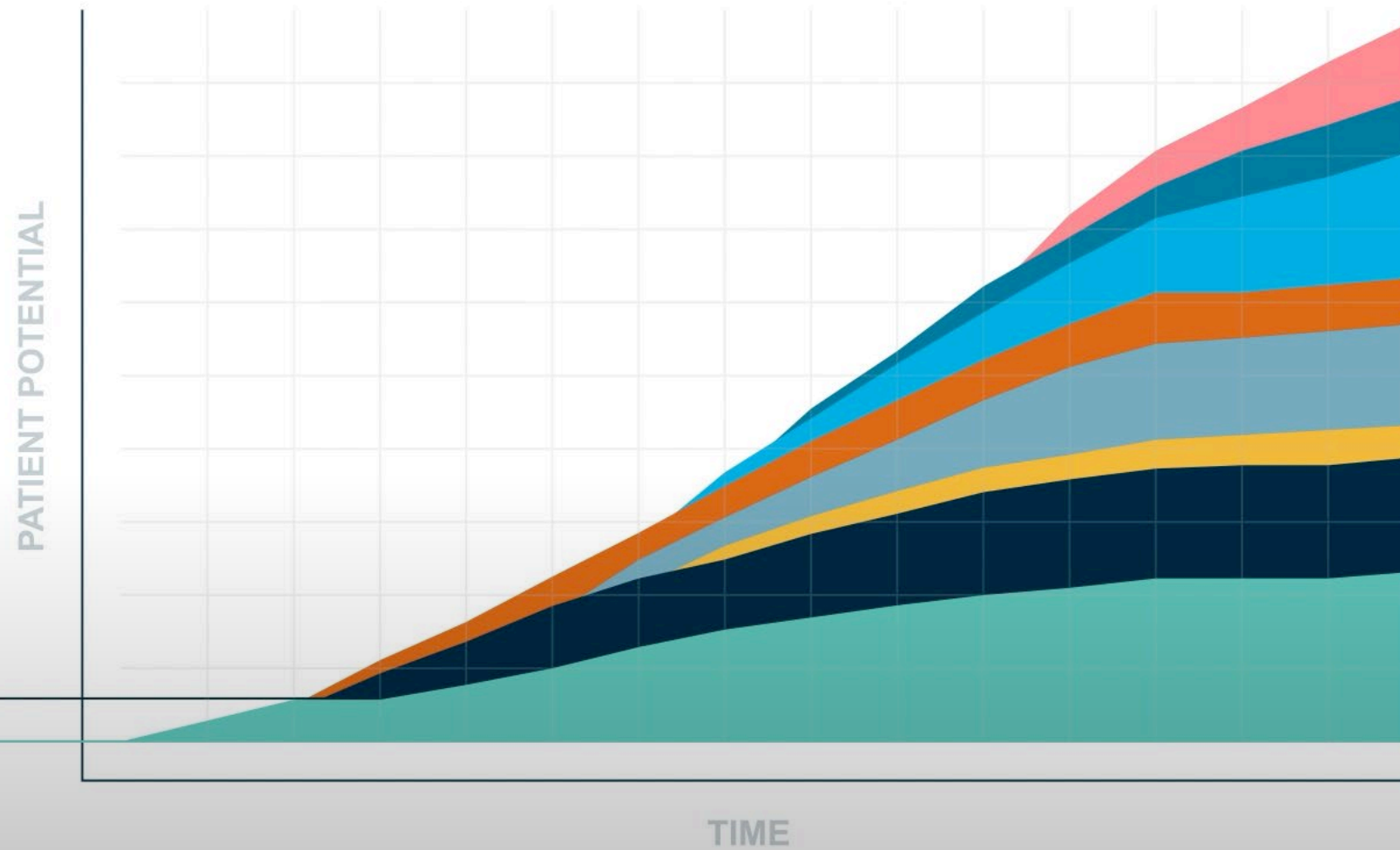
PMN



orladeyo
(berotralstat) capsules 150 mg



9930
orladeyo
(berotralstat) capsules 150 mg





CASH POSITION (IN MILLIONS)

Cash, Cash Equivalents, Restricted Cash & Investments at September 30, 2021	\$204
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Proforma – Cash, Cash Equivalents, Restricted Cash & Investments at September 30, 2021 ^A	\$548
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Senior Credit Facility at September 30, 2021 ^B	\$138
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A - Reflects approximate net cash received in November 2021 from Royalty Pharma and OMERS Capital Markets following transaction-related fees

B - From Athyrium Capital Management, term loan of \$125M interest-only for 5-year term, \$12.9M in interest payment-in-kind (PIK) has been added to principal since issuance. Does not reflect an additional \$75 million under the credit facility, which the company plans to draw in the middle of 2022





