Third Quarter 2021 Results Call
Corporate Update & Financial Results

November 3, 2021

Forward-Looking Statements

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Agenda

- Corporate Update:
  - Jon Stonehouse – President and Chief Executive Officer
- ORLADEYO® (berotralstat) Launch Update:
  - Charlie Gayer – Chief Commercial Officer
- Clinical Update
  - Dr. Helen Thackray – Chief Research and Development Officer
  - Dr. Bill Sheridan – Chief Medical Officer
- Financial Update
  - Anthony Doyle – Chief Financial Officer
- Summary and Q&A

Significant Milestones in 2021

Q12021

- **Approval** decision on ORLADEYO in Japan
- **Data** from completed BCX9930 dose ranging study in PNH

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

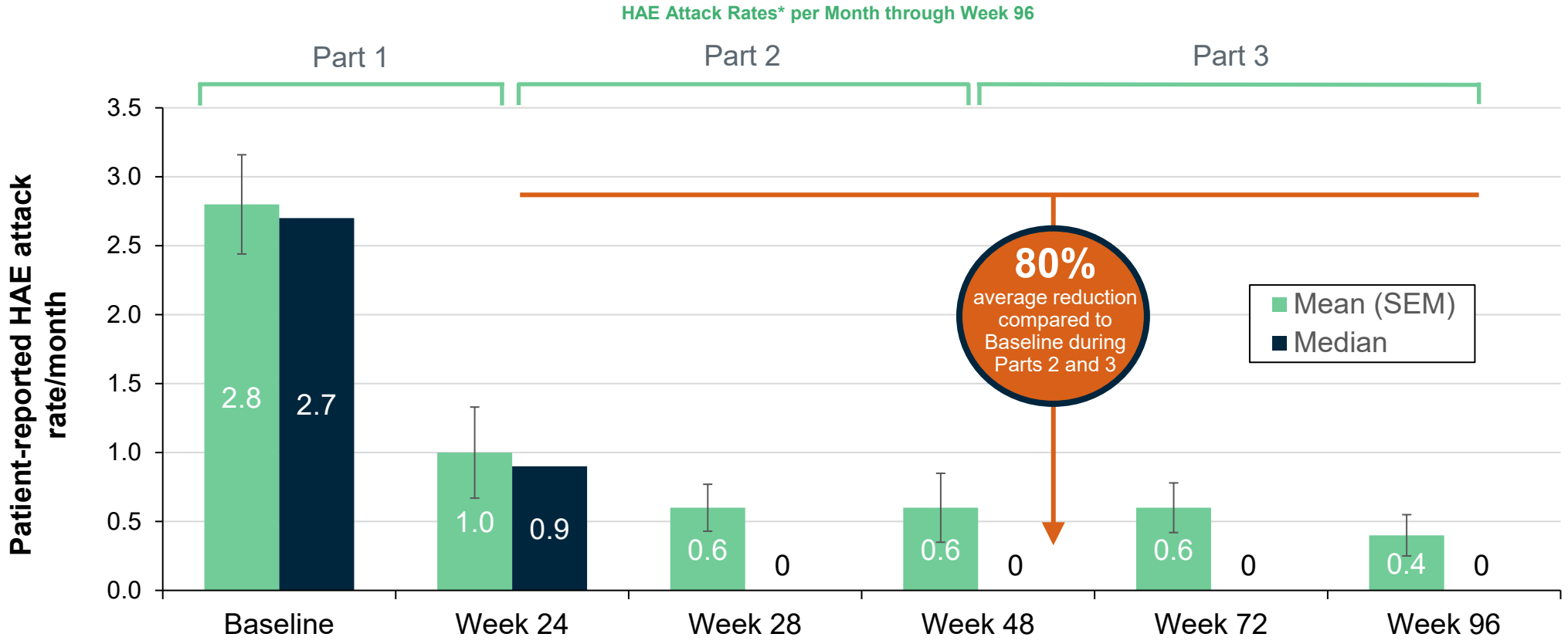
2H 2021

- **Begin Patient Enrollment in REDEEM-1 and REDEEM-2**
- **Begin Patient Enrollment in BCX9930 Renal PoC Trial**
- **Additional Global ORLADEYO Approvals/Launches**

ORLADEYO REVENUES

96 Week Data Highlights Efficacy of ORLADEYO

- In the patients who completed 96 weeks of treatment with berotralstat 150 mg (n=21), an 80% average reduction from the mean baseline attack rate per month was observed during Parts 2 and 3
- Median attack rates decreased over time from 2.7 attacks/month at baseline to 0.0 attacks/month in 16 of 17 months during Parts 2 and 3 (median 1.0 attacks/month in 1 month)



*Due to study design, investigator-confirmed attack rates were reported only for Parts 1 and 2, while subject-reported attack rates were reported for Part 3. For consistency across the entire 96 weeks, only subject-reported attack rates are reported. For analysis purposes, 1 month was defined as 4 weeks of treatment
Reasons for discontinuation in Part 3 include: perceived lack of efficacy (n=1), pregnancy (n=1), laboratory abnormality or adverse event (n=1), subject withdrew consent (n=2).
SD, standard deviation; SEM, standard error of the mean

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)

PNH Proof-of-Concept Study Long-term Follow-up

- **As of end of September, average dosing duration now exceeds 11 months, with the longest duration 19 months**

- **Data continues to support safety and tolerability of BCX9930, with no safety signals observed**

- **Update on patients naïve to C5-inhibitors, n=9**
  - All 9 patients naïve to C5-inhibitors continue to benefit from BCX9930
  - Hb responses have been maintained
  - No RBC transfusions have been needed during dosing at 400 mg or 500 mg BID BCX9930
  - Improvement in biomarkers of hemolysis and PNH RBC clone size have been maintained
  - No discontinuations from the trial

- **Update on patients with inadequate response to C5 inhibitors, n=6**
  - 3 patients remain in long-term follow-up – 2 transitioned to BCX9930 monotherapy
  - 3 patients have discontinued long-term follow-up, all unrelated to BCX9930:
    - 1 patient with very large transfusion burden pre-trial associated with pre-existing hypersplenism and other medical conditions complicating PNH*
    - 2 of 4 patients who had transitioned to BCX9930 monotherapy:
      - 1 developed illnesses unrelated to PNH
      - 1 for personal reasons

- **The safety and efficacy data collected from these patients in the long-term safety trial provides the company with a high degree of confidence for the success of the pivotal trials**

**As previously disclosed in August 2021*

Key Trial Endpoints and Statistical Analysis Approach

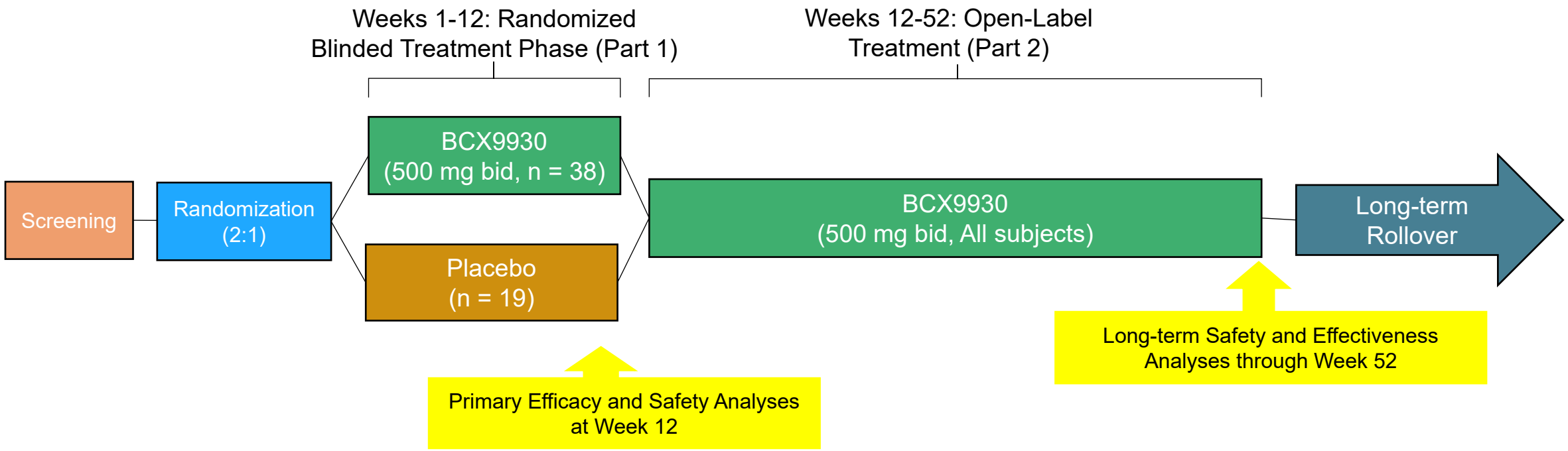
REDEEM 1 Transforming PNH Treatment

REDEEM 2 Transforming PNH Treatment

Primary Endpoint	Change from Baseline (CFB) in hemoglobin (Hb) [mean of Weeks 12, 16, 20, 24]	CFB in Hb [Week 12]
Key Secondary Endpoints	Proportion of subjects who are transfusion free [Day 14 to Week 24]	Proportion of subjects who are transfusion free [Day 14 to Week 12]
	Number of units of packed red blood cells (RBC) transfused [Day 14 to Week 24]	Number of packed RBC units transfused [Day 14 to Week 12]
	CFB in Functional Assessment of Chronic Illness Therapy (FACIT)-Fatigue scale score [mean of Weeks 12, 16, 20, 24]	Percent CFB in lactate dehydrogenase (LDH) [Week 12]
		CFB in FACIT-Fatigue scale score [Week 12]
ITT Analysis* of Primary Endpoint	Analysis of Covariance (ANCOVA)	ANCOVA

** In each trial, multiplicity is controlled by hierarchical testing of primary and then key secondary endpoints in the order listed in the table*

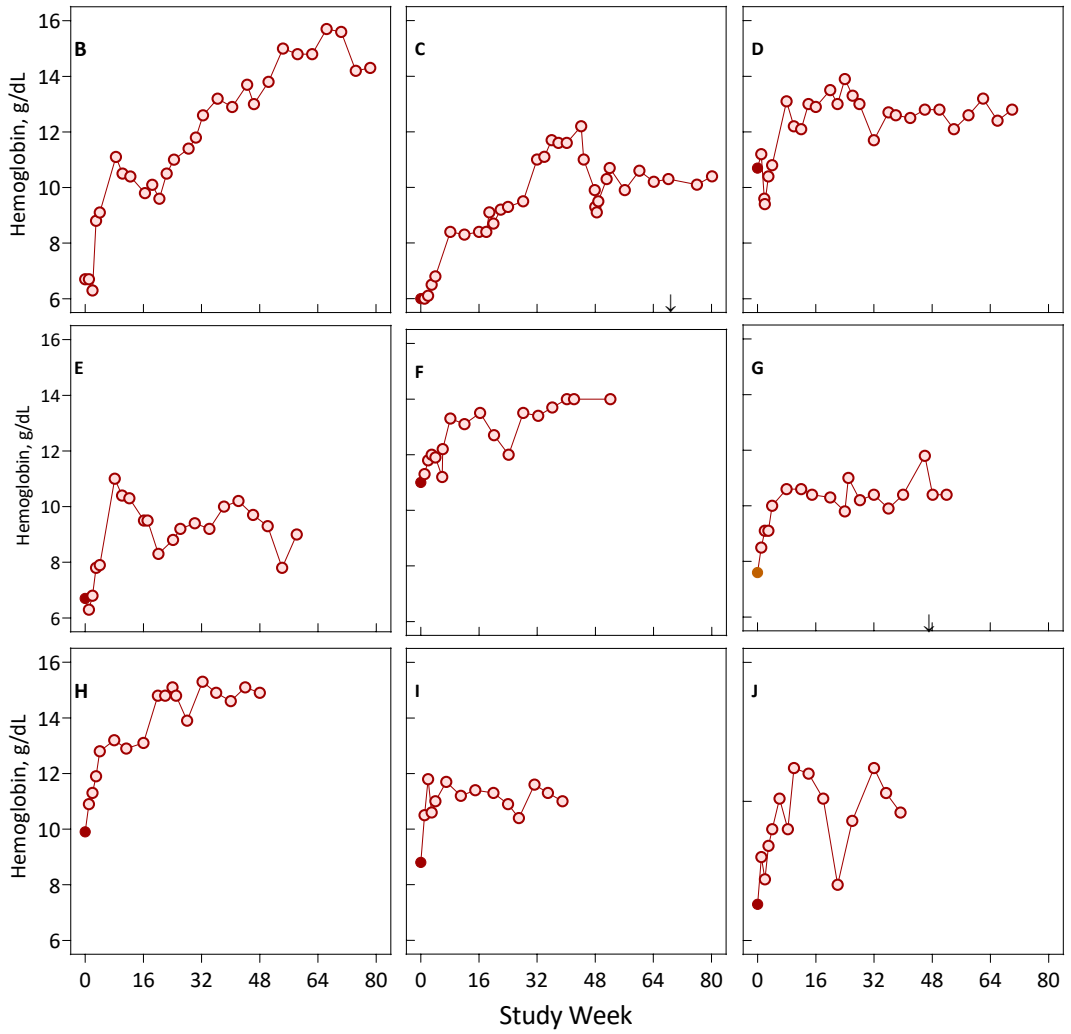
Pivotal Trial of BCX9930 as Oral Monotherapy in PNH Patients not Currently Receiving C5 Inhibitor Therapy



- Key eligibility criteria include screening Hb ≤ 10.5 g/dL, reticulocyte count ≥ 100,000/µL, and LDH ≥ 2 × upper limit of normal
- Randomization is stratified by RBC transfusion (yes vs. no) within the 6 months prior to baseline
- REDEEM-2 is powered at 90% to detect a difference in mean change from baseline of hemoglobin of ≥ 2.15 g/dL

Key parameters in C5-INH naïve patients (n=9)

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)

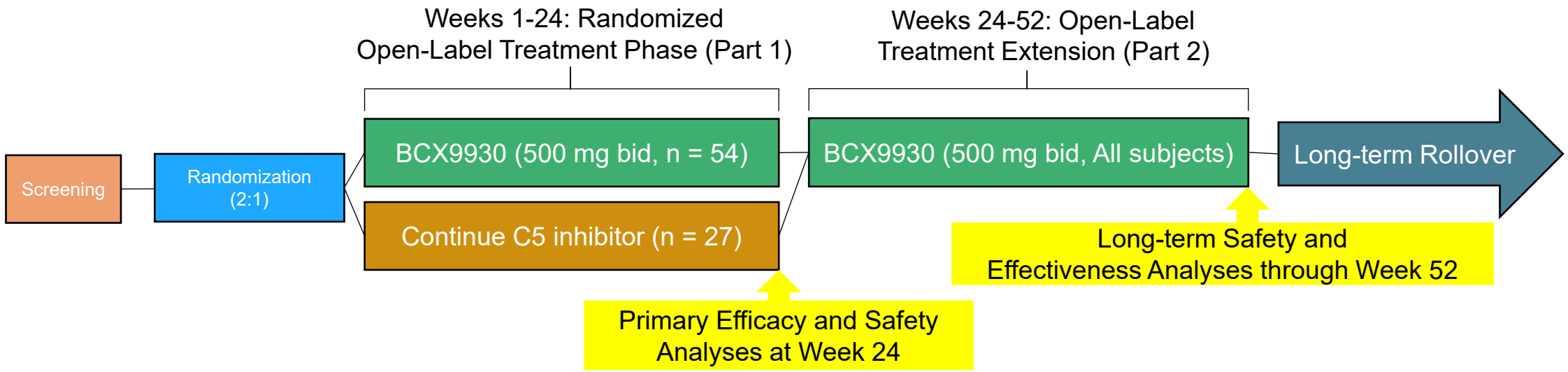
REDEEM-2 is powered at 90% to detect a difference in mean change from baseline of hemoglobin of 2.15 g/dL

Note: For calculation of CFB, averaged valid pre-dose values determine the Hb baseline value and the latest pre-dose value determines the LDH and FACIT baseline value

**As disclosed in May 2020, Patient A (not shown) discontinued due to an unrelated SAE*

§ Preliminary data

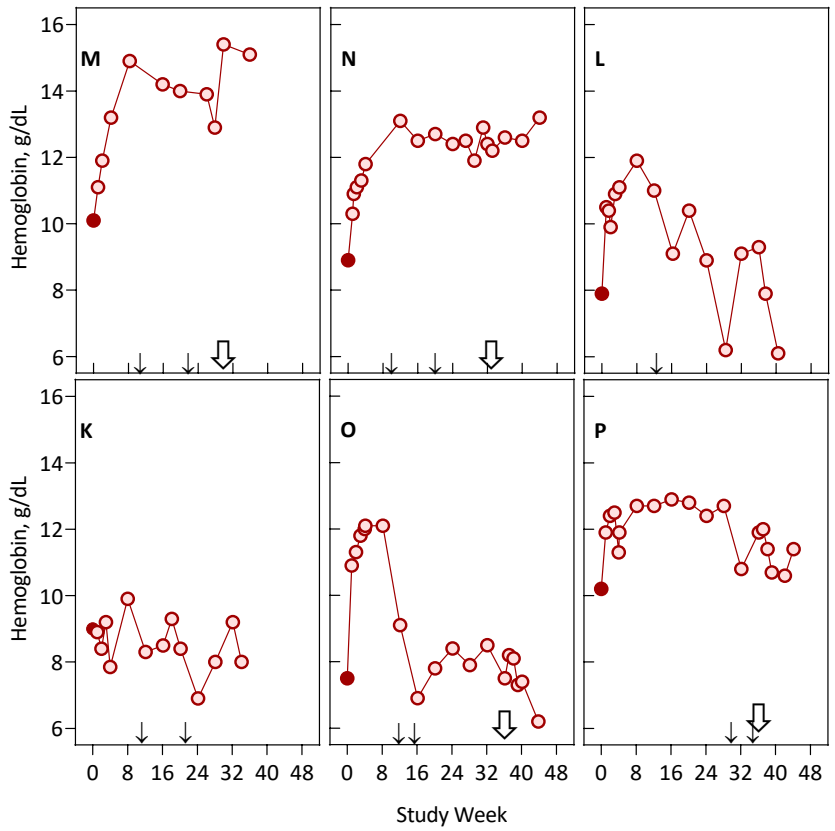
Pivotal Trial of BCX9930 as Oral Monotherapy in PNH Patients with Inadequate Response to C5-Inhibitor Therapy



- Key eligibility criteria include screening Hb ≤ 10.5 g/dL and reticulocyte count ≥ 100,000/µL on a stable regimen of eculizumab or ravulizumab
- Randomization is stratified by: C5 inhibitor (ravulizumab vs. eculizumab); and RBC transfusion (yes vs. no) within the 6 months prior to baseline
- REDEEM-1 is powered at 90% to detect a difference in mean change from baseline of hemoglobin of ≥ 2 g/dL

Key parameters in C5-INH inadequate response patients (n=6)

Hemoglobin by Patient, BCX9930 POC Study



⇩ -- Start of BCX9930 monotherapy
↓ -- COVID-19 Vaccine Doses
Top Row: Patients continuing on study
Bottom Row: Patients discontinued long-term follow-up:
K: preexisitng hypersplenism
O: devloped illnesses unrelated to PNH including COVID vaccination associated immune hemolytic anemia
P: personal reasons

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

Note: For calculation of CFB, averaged valid pre-dose values determine the Hb baseline value and the latest pre-dose value determines the LDH and FACIT baseline value.

§ *Preliminary data*

Cash position (in millions)

Cash, cash equivalents, restricted cash & investments at December 31, 2020	$303
Cash, cash equivalents, restricted cash & investments at September 30, 2021	$204
Senior credit facility [A]	$138

A – 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

Based on the strength of the ORLADEYO launch thus far, and continued growth from new patient demand expected in the fourth quarter, the company now expects net ORLADEYO revenue for full year 2021 to be between $115 million and $120 million. 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.

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