Second Quarter 2021 Results Call
Corporate Update & Financial Results

August 5, 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

- Financial Update

  Anthony Doyle – Chief Financial Officer

- BCX9930 Update

  Dr. Bill Sheridan – Chief Medical 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

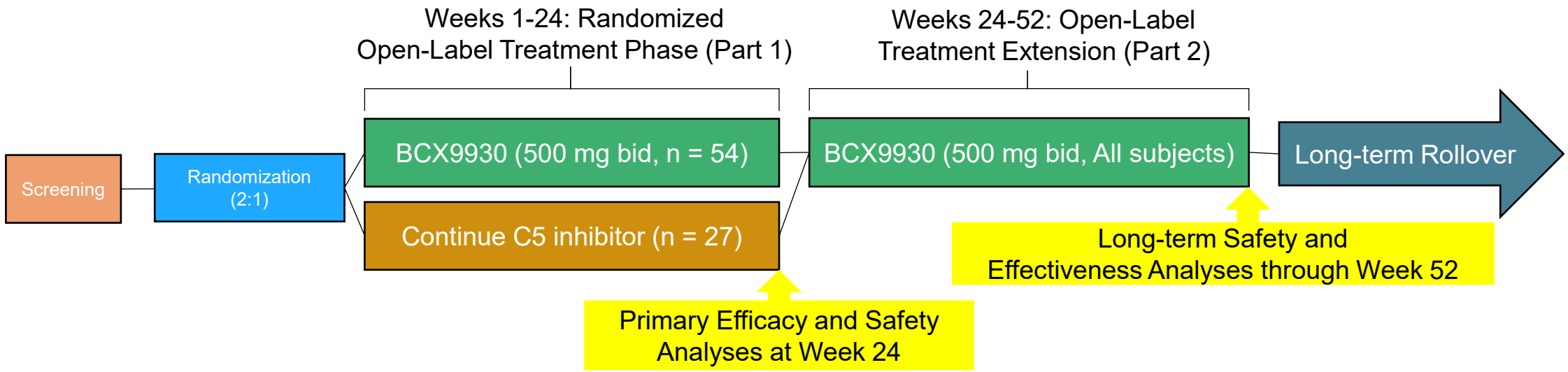
- **Approval** decision on ORLADEYO in EU
- **Revenues** reported from Q1/first full quarter of ORLADEYO sales in US
- **Launch** of ORLADEYO in Japan
- **Launch** of ORLADEYO in Germany

2H 2021

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

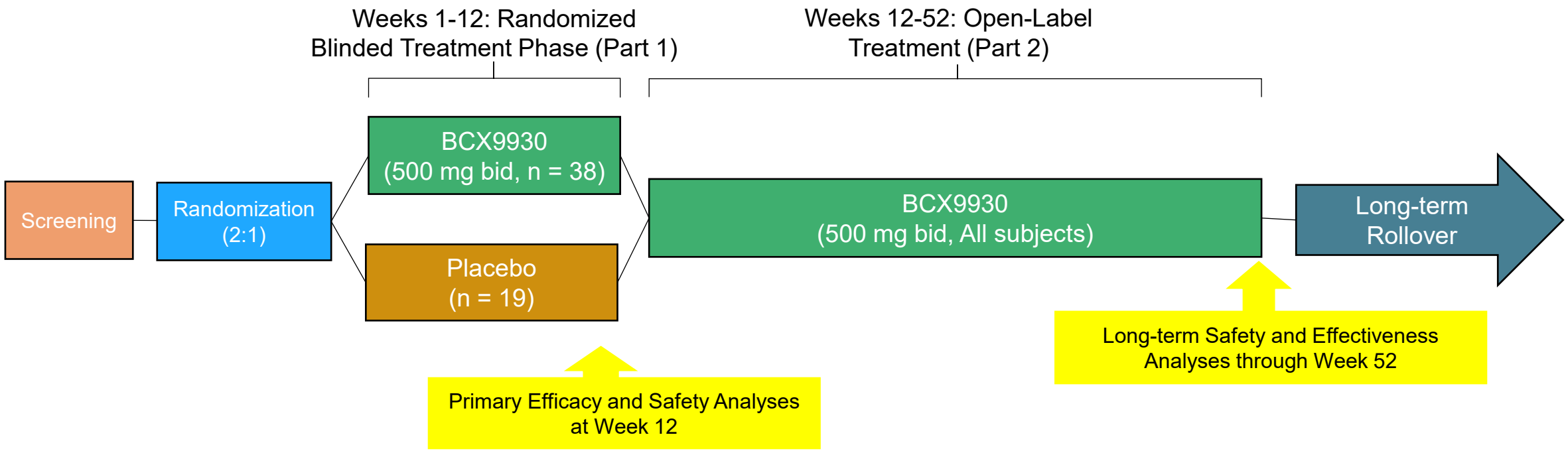
ORLADEYO REVENUES

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

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 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*

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

- **Average dosing duration now exceeds 9 months, with the longest duration 17 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**
  - 5 with high levels of C3 opsonization on PNH RBCs at baseline continue to benefit from BCX9930
    - So far, four patients have transitioned to BCX9930 monotherapy, including one patient who had previously received RBC transfusions during hemolysis following COVID-19 vaccination while being treated with both ravulizumab and BCX9930
  - 1 patient with very large transfusion burden pre-trial associated with pre-existing hypersplenism and other medical conditions complicating PNH has discontinued from the trial
    - This patient continued to need RBC transfusions, although at a reduced frequency
    - This patient had very low PNH RBC C3 opsonization at baseline, indicating that complement-mediated hemolysis was likely not a main factor in this individual's anemia

Cash position (in millions)

Cash, cash equivalents, restricted cash & investments at December 31, 2020	$303
Cash, cash equivalents, restricted cash & investments at June 30, 2021	$223
Senior credit facility [A]	$132

A – From Athyrium Capital Management, term loan of $125M interest-only for 5-year term, $7.5M in interest payment-in-kind (PIK) has been added to principal since issuance

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.

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