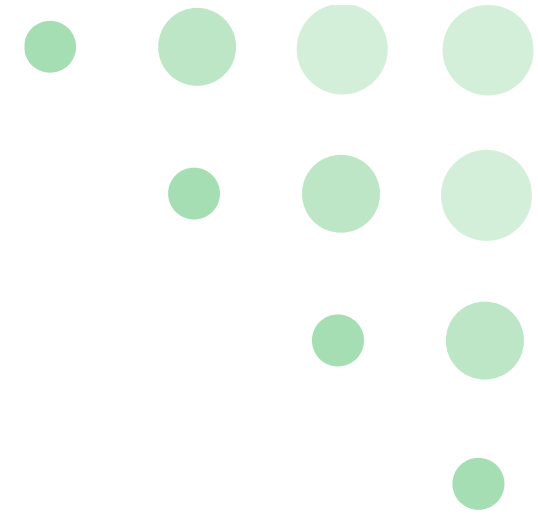


# Fourth Quarter 2021 Results Call Corporate Update & Financial Results

February 23, 2022



# 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 <https://ir.biocryst.com/financial-information/sec-filings>.

# Agenda

- ◆ Corporate Update:

Jon Stonehouse – President and Chief Executive Officer

- ◆ ORLADEYO<sup>®</sup> (berotralstat) Launch Update:

Charlie Gayer – Chief Commercial Officer

- ◆ Clinical Update

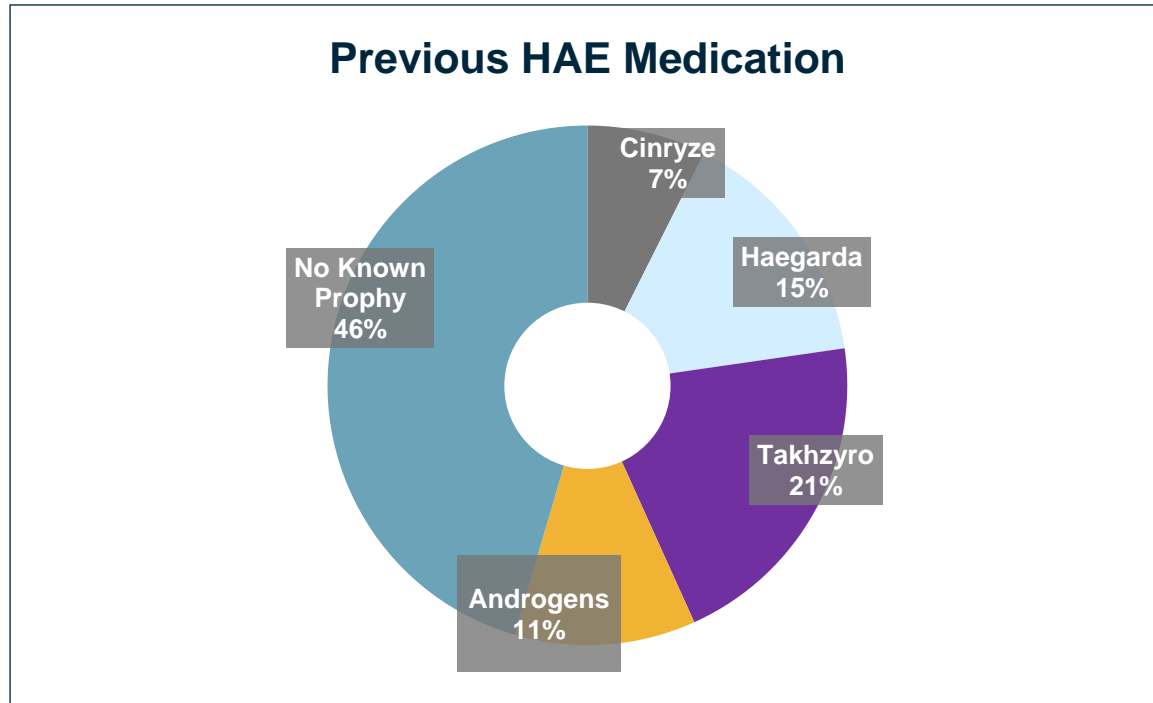
Dr. Helen Thackray – Chief Research and Development Officer

- ◆ Financial Update

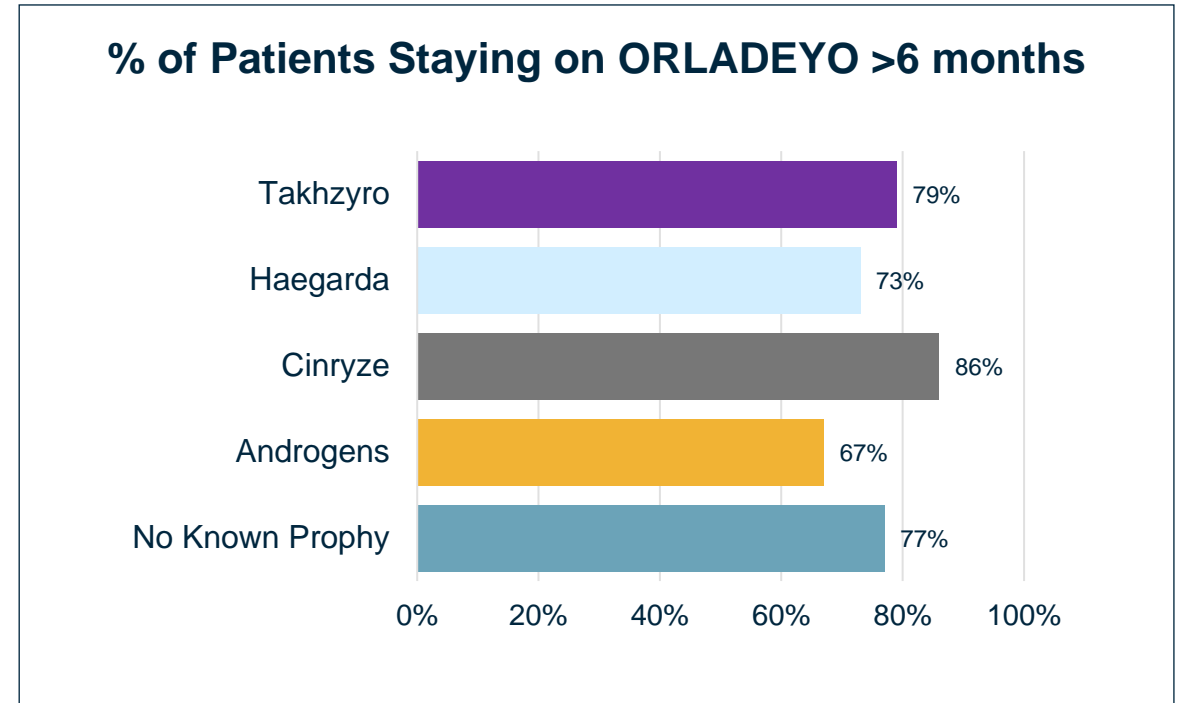
Anthony Doyle – Chief Financial Officer

- ◆ Summary and Q&A

# PATIENTS ON ORLADEYO: COMPARISON BY PREVIOUS HAE MEDICATION



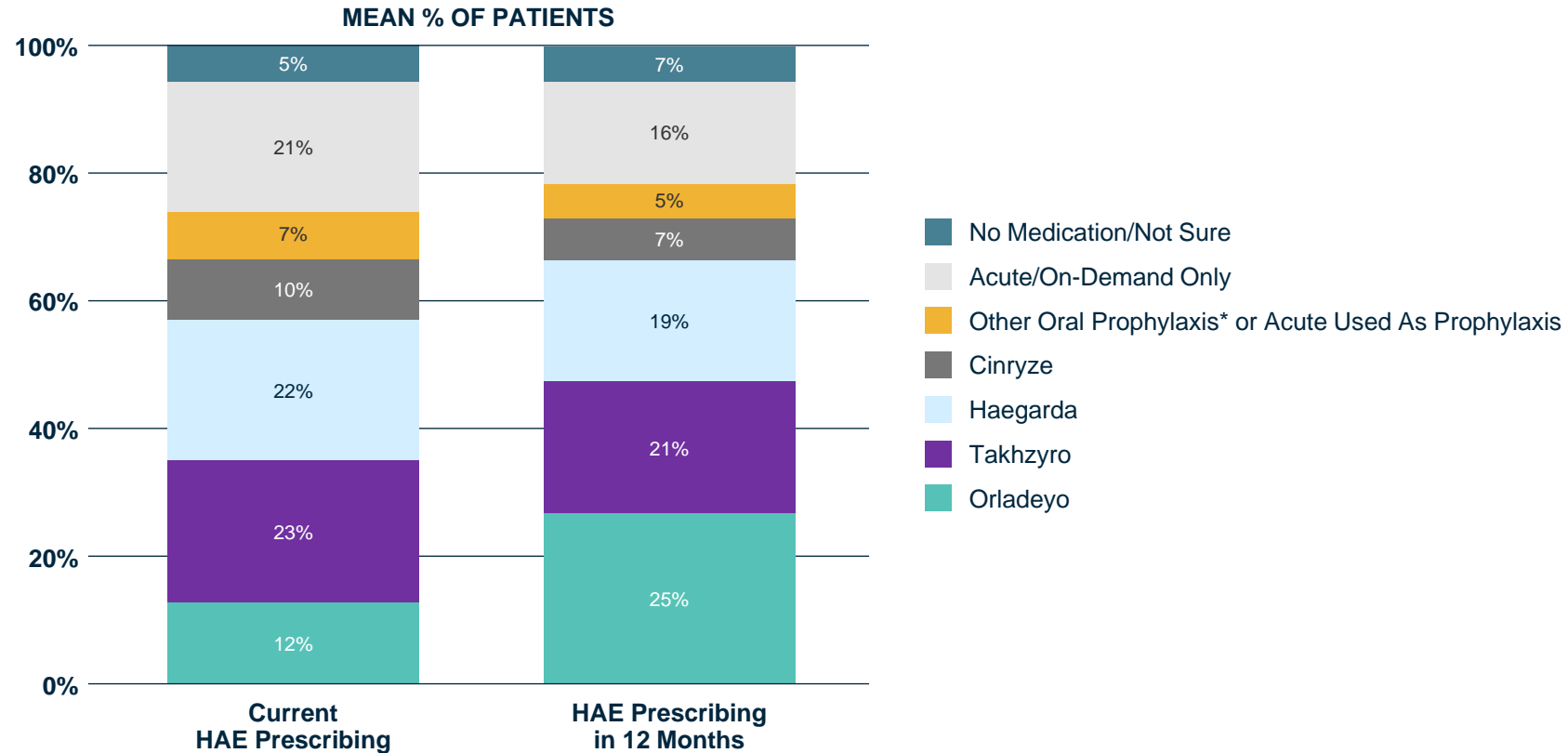
Source: Estimates from internal analysis of ORLADEYO patients starting therapy Dec'20-Nov'21, consented with medical history



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

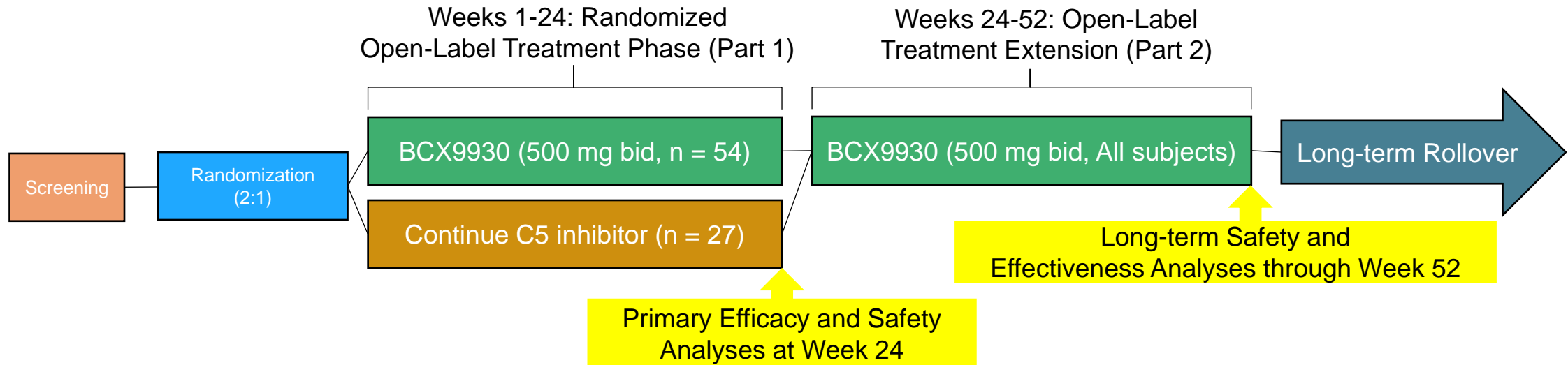
**Future Prescribing of HAE Medications for Prophylaxis (Current & In Next 12 Months)**  
*All Qualified Respondents (n=60)*



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

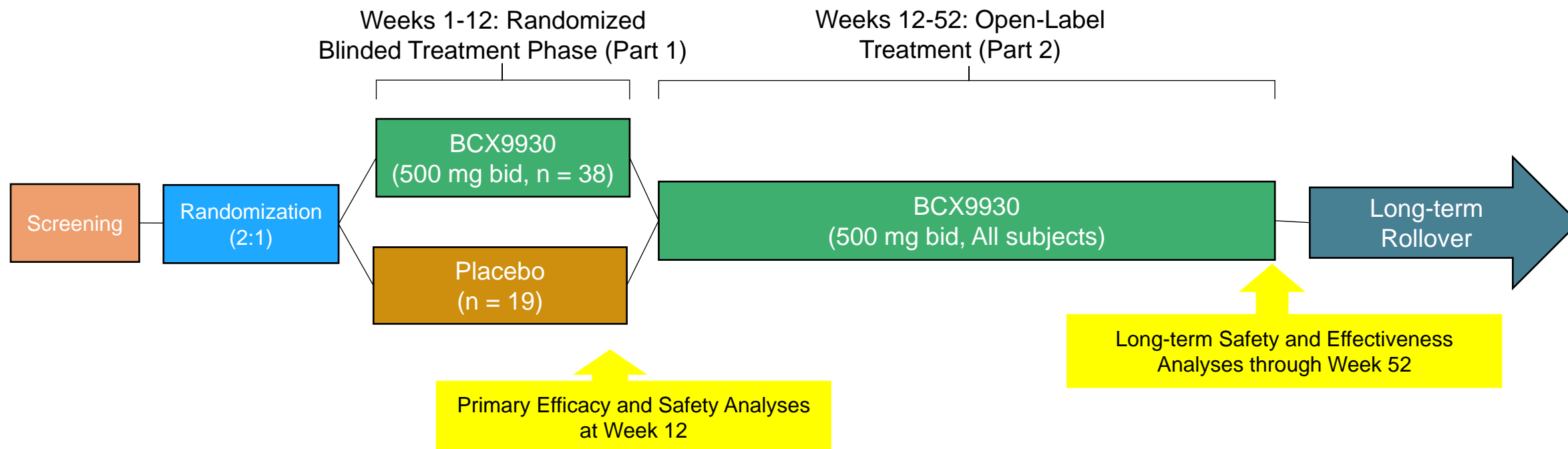
Source: BioCryst Proprietary Market Research conducted with 60 Allergist/Immunologists in August 2021

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



- Key eligibility criteria include screening Hb  $\leq$  10.5 g/dL and reticulocyte count  $\geq$  100,000/ $\mu$ L 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  $\geq$  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  $\leq$  10.5 g/dL, reticulocyte count  $\geq$  100,000/ $\mu$ L, and LDH  $\geq$  2  $\times$  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  $\geq$  2.15 g/dL

# Key Trial Endpoints and Statistical Analysis Approach

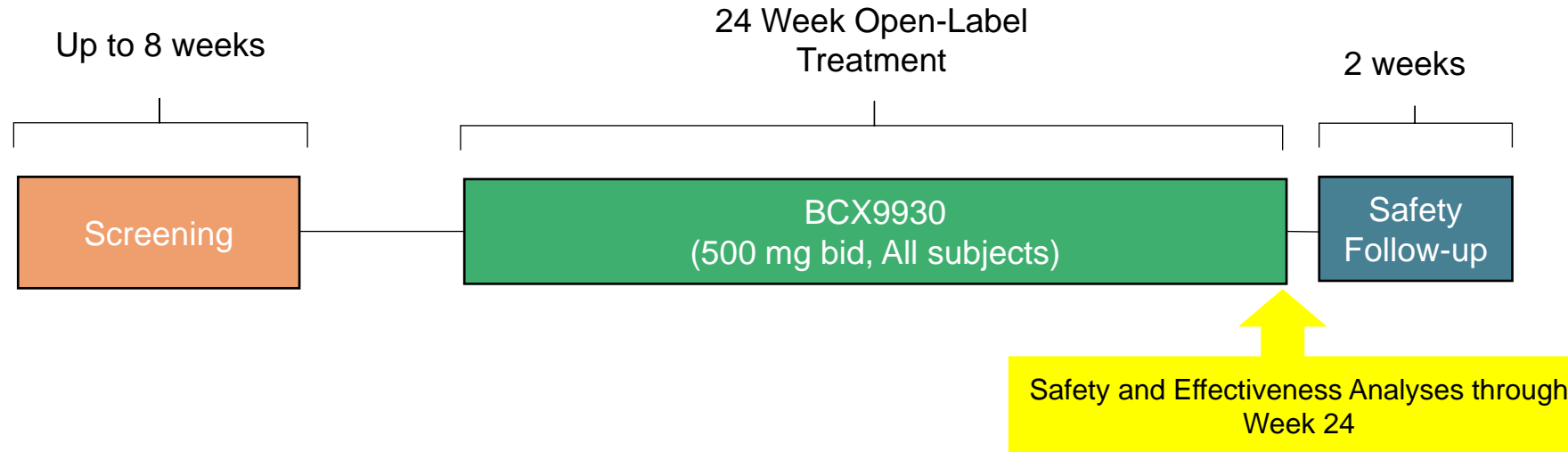


Primary Endpoint	Change from Baseline (CFB) in hemoglobin (Hb) [mean of Weeks 12, 16, 20, 24]	CFB in Hb [Week 12]
Key Secondary Endpoints	Proportion of subjects who are transfusion free [Day 14 to Week 24]	Proportion of subjects who are transfusion free [Day 14 to Week 12]
	Number of units of packed 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



# Proof-of-Concept Trial of BCX9930 as Oral Therapy in Patients with C3G, IgAN or PMN



- Primary diagnosis of C3G, IgAN, or PMN confirmed by central pathology review of digital images and pathology reports of renal biopsy samples obtained during screening
- Enrollment will include a total of approximately 42 adult subjects (up to 14 each for the 3 included diseases, C3G, IgAN, or PMN) who have persistent proteinuria despite receiving a maximally tolerated dose of an angiotensin-converting enzyme inhibitor or angiotensin receptor blocker

# Key Trial Endpoints



Primary Endpoint	Change in 24-hour urinary protein excretion normalized to urine creatinine (uPCR)
Key Secondary Endpoints	Proportion of subjects with a uPCR response: <ul style="list-style-type: none"> <li>• Partial remission, <math>\geq 50\%</math> reduction from baseline</li> <li>• Complete remission, <math>\leq 500</math> mg/g</li> <li>• Normalization, <math>\leq 200</math> mg/g</li> </ul>
	Change from baseline in eGFR
	Number and proportion of subjects with a morphologic response on renal biopsy
	Change from baseline in blood and urine levels of complement biomarkers

# Cash position (in millions)

Cash, cash equivalents, restricted cash & investments at December 31, 2020	\$303
Cash, cash equivalents, restricted cash & investments at December 31, 2021	\$518
Senior credit facility <sup>A</sup>	\$142
<b>FY 2022 GUIDANCE</b>	
ORLADEYO Revenue	> \$250
Operating expenses <sup>B</sup>	\$440 - \$480

A – From Athyrium Capital Management, term loan of \$125M interest-only for 5-year term, \$17.1M in interest payment-in-kind (PIK) has been added to principal since issuance

B – Excludes equity-based compensation.

# Fourth Quarter 2021 Results Call Corporate Update & Financial Results

February 23, 2022

