Third Quarter 2020 Results Call Corporate Update & Financial Results

November 5, 2020



Forward-Looking Statements

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Agenda

Corporate Update:

Jon Stonehouse – President and Chief Executive Officer

◆ ORLADEYO[™] (berotralstat) Launch Readiness:

Charlie Gayer – Chief Commercial Officer Megan Sniecinski – Chief Business Officer

BCX9930 Update

Dr. Bill Sheridan – Chief Medical Officer

Financial Update

Anthony Doyle – Chief Financial Officer

Summary and Q&A

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Corporate Update: Jon Stonehouse President and Chief Executive Officer

Multiple Upcoming Program Updates

Q4 2020	PDUFA Dec. 3, 2020			Q1 2021	Q2 2021
Data from part 1 galidesivir COVID-19 trial (Q4 2020)	ORLADEYO approval in U.S.	Approval decision on ORLADEYO in Japan (December 2020)	Phase 1 data from BCX9250 trial in FOP (Year-end 2020)	Data from completed BCX9930 dose ranging study in PNH (Q1 2021)	Approval decision on ORLADEYO in EU (Q2 2021)

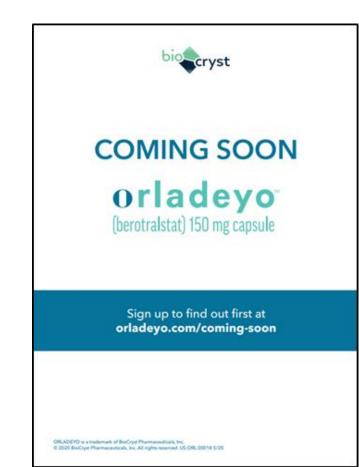
ORLADEYO Approvals Expected in U.S., Japan, EU



ORLADEYO[™] (berotralstat) Launch Readiness: Charlie Gayer – Chief Commercial Officer Megan Sniecinski – Chief Business Officer

Coming Soon: Orladeyo™

Orladeyo[™] (berotralstat) 150 mg capsule





Recent and Upcoming Publications Support Launch

APeX-2 Trial



American College of Allergy, Asthma & Immunology (ACAAI) 2020 Annual Scientific Meeting

November 13-15, 2020

Distinguished Industry Oral Abstract:

Berotralstat Reduces Attacks in Patients with Hereditary Angioedema (HAE): APeX-2 Trial 48 Week Results

Posters:

- Berotralstat Improves Patient-Reported Quality of Life Through 48 Weeks in the Phase 3 APeX-2 Trial
- Berotralstat Positively Impacts Patient-Reported Satisfaction: Results from the Phase 3 APeX-2 trial
- Patient Perspectives on the Treatment Burden of Injectable Medication Administration for Hereditary Angioedema
- Prophylactic Treatment Burden: Assessment by Caregivers of Patients with Hereditary Angioedema
- Understanding Differences in Perceptions of Hereditary Angioedema Treatment Burden May Improve Patient-Physician Treatment Care Dialogue

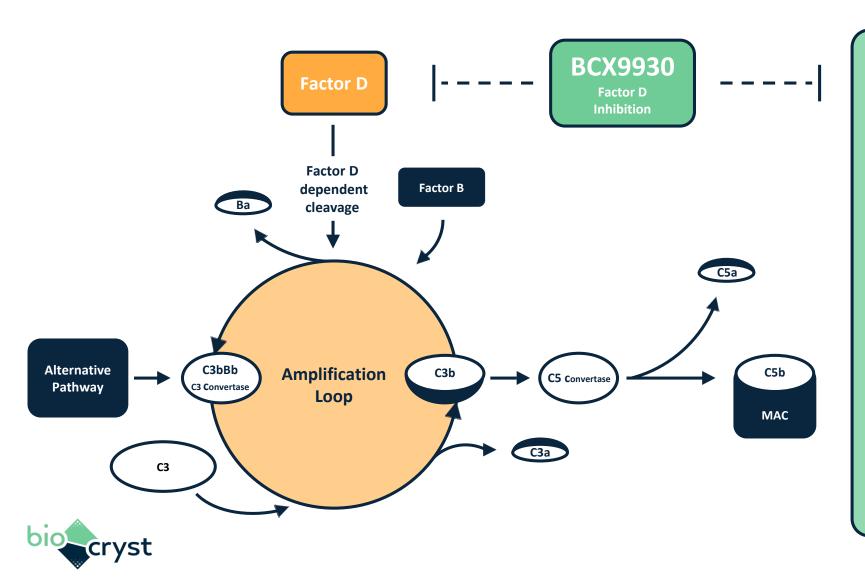


Clinical Update: Dr. Bill Sheridan – Chief Medical Officer

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

•Factor D is essential to initiate the Alternative Pathway

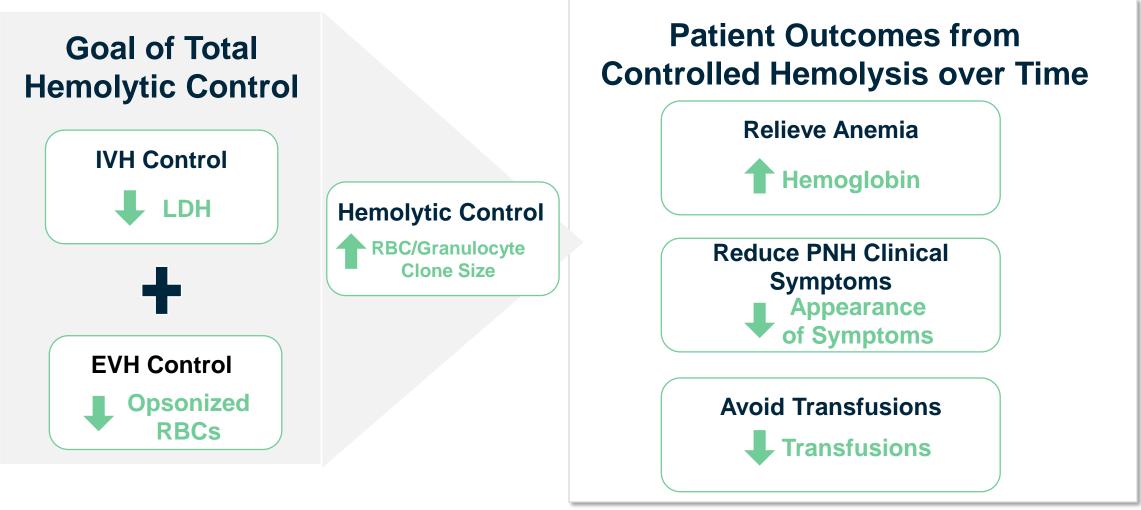
•Blocking Factor D blocks the Alternative Pathway and all downstream products



Dysregulation Diseases Hematology Rheumatology **PNH ANCA** vasculitis paroxysmal nocturnal antineutrophil hemoglobinuria cytoplasmic antibodyassociated vasculitis aHUS atypical hemolytic Lupus nephritis uremic syndrome IgAN vasculitis Nephrology C3G glomerulonephritis **PMN** primary membranous nephropathy

Spectrum of Alternative Pathway

IgAN *IgA nephropathy* Oral Monotherapy with BCX9930 Offers Advantage of Controlling Both Intravascular (IVH) and Extravascular (EVH) Hemolysis





Financial Update: Anthony Doyle– Chief Financial Officer

Cash position & 2020 guidance (in millions)

Cash, cash equivalents, restricted cash & investments at December 31, 2019	\$138				
Cash, cash equivalents, restricted cash & investments at September 30, 2020	\$149				
Senior credit facility ^A	\$46				
REVISED FY 2020 GUIDANCE					
REVISED FY 2020 GUIDANCE					
REVISED FY 2020 GUIDANCE Net operating cash utilization	\$150-165				

A - Excludes equity-based compensation.



Thank You... Questions and Answers

