Fourth Quarter 2020 Results Call
Corporate Update & Financial Results

February 25, 2021

Forward-Looking Statements

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Agenda

- Corporate Update:
  - Jon Stonehouse – President and Chief Executive Officer
- Global ORLADEYO™ (berotralstat) Launches:
  - 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

Corporate Update:

Jon Stonehouse

President and Chief Executive Officer

Significant Upcoming Milestones in 2021

Q12021

- **Approval** decision on ORLADEYO in Japan (January 2021)
- **Data** from completed BCX9930 dose ranging study in PNH (R&D Day: March 22)

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

Q32021

BCX9930 Advanced Development Trials

BCX9250 Next Steps

Q42021

ORLADEYO REVENUES

Global ORLADEYO™ (berotralstat) Launches:

Charlie Gayer – Chief Commercial Officer

Megan Sniecinski – Chief Business Officer

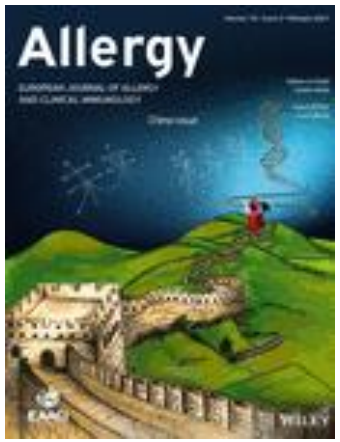
Now Available: Orladeyo™

orladeyo™
(berotralstat) capsules 150 mg

Recent and Upcoming Publications Support Launch

APeX-2 Trial

APeX-J Trial



2021 American Academy of Allergy, Asthma & Immunology (AAAAI) Annual Meeting

February 26-March 1, 2021.

Oral Abstract Presentation:

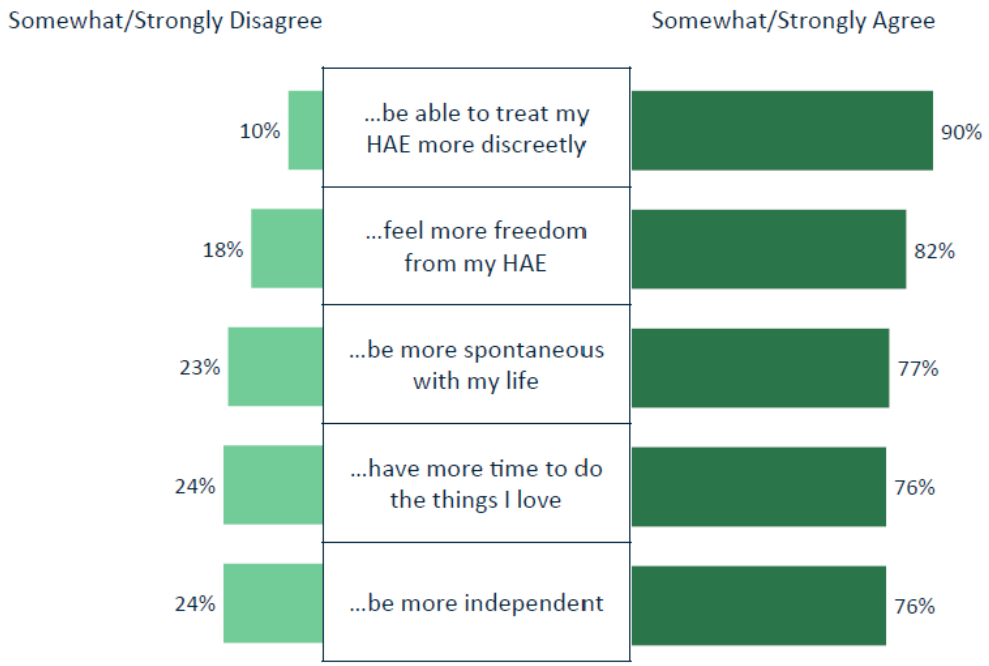
- *Berotralstat Reduces Use of On-demand Medication in Hereditary Angioedema (HAE) Patients Previously Treated with Prophylactic Therapies*

Posters:

- *Chart Audit Study: Physician-Reported Hereditary Angioedema Attacks for Patients on Prophylactic Treatments*
- *Berotralstat Consistently Demonstrates Reductions in Attack Frequency in Hereditary Angioedema (HAE) Irrespective of Baseline Attack Rate: Subgroup Analysis from the APeX-2 Trial*
- *Reduction in Attacks in Hereditary Angioedema (HAE) with Berotralstat is Consistent Regardless of Prior Prophylactic Treatment: A Subgroup Analysis of the Phase 3 APeX-2 Trial*

Significant Burden of Treatment Reported by Patients, Caregivers, and Treating Physicians

Patient perspective:
If I were prescribed a once daily pill to prevent (prophylaxis) HAE attacks, I would…*

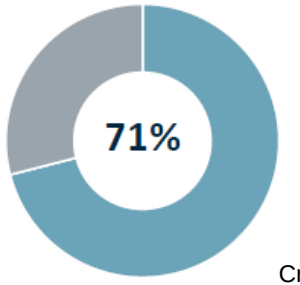


Agreement with statements regarding a once-daily oral HAE medication (n = 75)

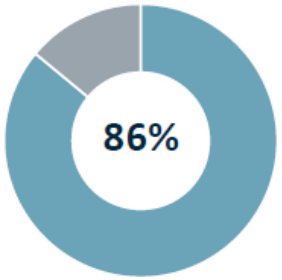
Radojicic et al., ACAAI 2020, Poster #160

Caregivers perspective: How patients perceive their prophylactic treatment

[Patient] is tired of his/her infusions/injections
*% of caregivers who somewhat/strongly agree**

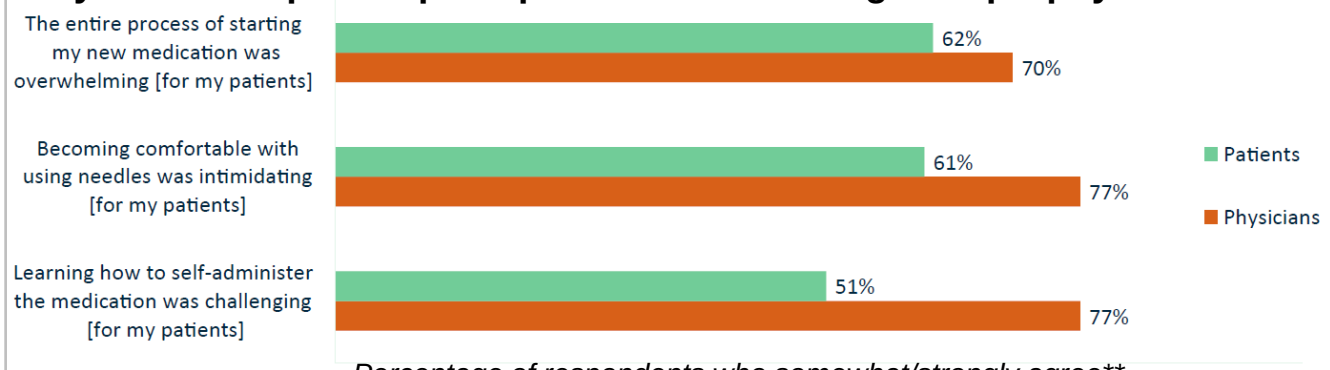


[Patient] is satisfied with his/her current HAE treatment, but would still be interested in one that is easier to administer
*% of caregivers who somewhat/strongly agree**



Craig et al., ACAAI 2020, Poster #161

Physician and patient perceptions about starting HAE prophylaxis:



*Percentage of respondents who somewhat/strongly agree***

Riedl et al., ACAAI 2020, Poster #162

Cross-sectional study conducted via three double-blinded surveys with HAE patients (n=75), caregivers (n=30) and physicians (n=109)

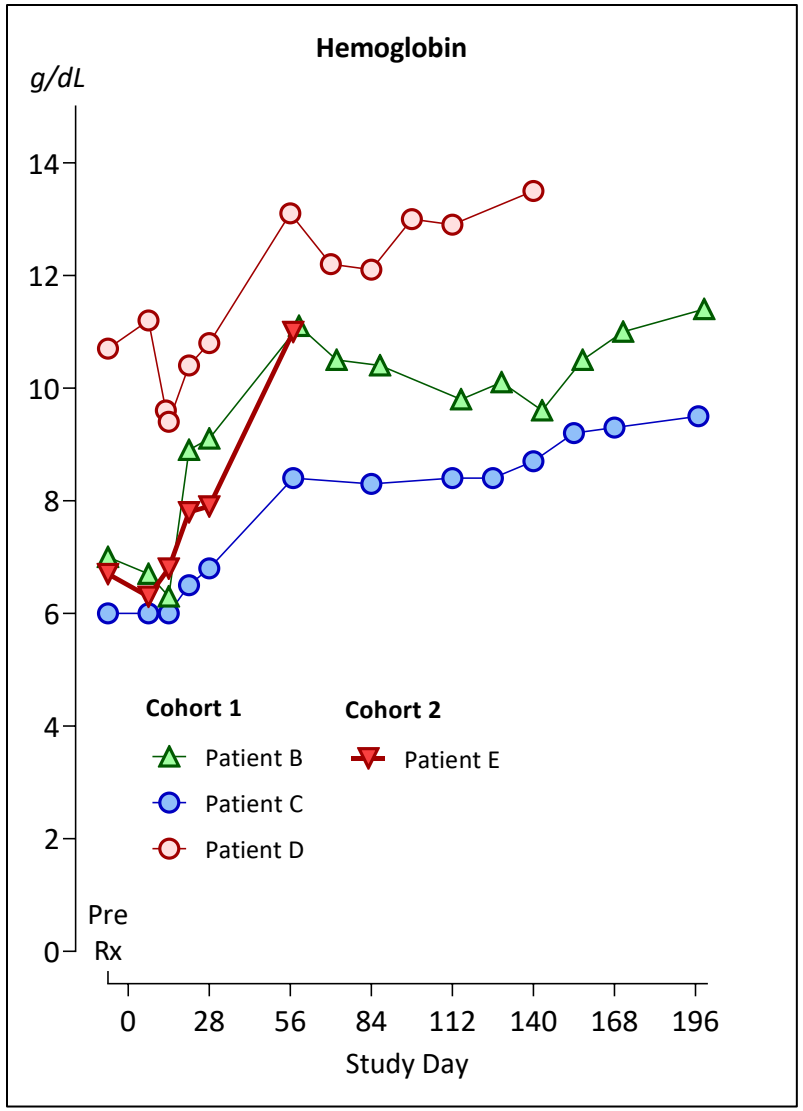
**Bases vary; not applicable/not sure excluded;*

***Bases vary; not applicable/not sure excluded; text in brackets was included in the response options for physicians*

Clinical Update:

Dr. Bill Sheridan – Chief Medical Officer

Meaningful Changes in Key Biomarkers Indicating Control of Hemolysis



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***

- 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

Study is ongoing – preliminary data as reported 9/30/20. One 2-unit RBC transfusion in Patient B on study day 15 after 50 mg BID x 14 d (previously reported).

BCX9930 has been Safe and Well Tolerated in PNH Patients

Overall Safety

- No discontinuations due to related AEs
- No BCX9930-related serious AEs or safety signals
- No safety signals in routine monitoring of vital signs, ECGs, or laboratory evaluations of hematology, coagulation, urinalysis, or clinical chemistry

Adverse Events

- The most common drug-related TEAE was mild-moderate headache lasting 1-3 days
- Two patients had mild rash that resolved during continued uninterrupted BCX9930 dosing
- One unrelated serious AE*

Study is ongoing – preliminary data as reported 9/30/20.

**Unrelated SAE previously reported, primary disseminated VZV infection in a non-immune subject taking corticosteroids, fatal.*

Q1 Data Readout and Next Steps for BCX9930

Phase 1 dose-ranging trial in PNH has fully enrolled

- Data from total of 16 patients
- Duration of treatment up to >48 weeks
- Both treatment-naïve patients (n=10) and C5 inadequate responders (n=6)
- Data from 15 patients treated at doses of 400 mg bid or 500 mg bid for at least six weeks
- Data to be announced at R&D Day on March 22nd
    - Plan to report range of clinical and laboratory outcomes, biomarkers and safety data

Next Steps

- Begin (2H 2021) pivotal trials in PNH patients at selected dose level
- Begin (2H 2021) PoC trial(s) in patients with renal complement-mediated diseases at same selected dose

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

Financial Update:

Anthony Doyle– Chief Financial Officer

Cash position (in millions) and 2021 Financial Outlook

Cash, cash equivalents, restricted cash & investments at December 31, 2019	$138
Cash, cash equivalents, restricted cash & investments at December 31, 2020 [A]	$303
Senior credit facility [B]	$125

A – Reflects net cash received in December 2020 from Royalty Pharma and Athyrium Capital Management following transaction-related fees and payoff of prior MidCap debt

B - From Athyrium Capital Management, $125M interest-only for 5-year term

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.

Thank You…
Questions and Answers