June 2020
Corporate Presentation
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Coming Soon: Orladeyo™

(berotralstat) 150 mg capsule
Rapid and Sustained HAE Attack Rate Reduction: Analyses for APeX-2: 150 mg and Placebo 48-week Completers

**APeX-2 Attack Rate by Month, Completers Analysis**

BCX7353 150 mg QD

- BCX7353 150 mg, n=30

Placebo and Placebo-to-Active

- Placebo, n=27
- BCX7353 150 mg after placebo, n=13
- BCX7353 110 mg after placebo, n=14
Consistent Mean Attack Rates in APeX-2 and APeX-S

APeX-2 and APeX-S Completers Analyses, 150 mg QD BCX7353

Mean (SEM) attack rate per 4-week month

APeX-2 150 mg QD, n=30
APeX-S 150 mg QD, n=73
Median Attack Rates in 48-week Completers:
Zero Attacks per Month in 6 of 12 Months in APeX-S
Safety and Tolerability Confirmed in Integrated 48-week Analysis

### Subjects enrolled and dosed [Safety Population]

<table>
<thead>
<tr>
<th></th>
<th>BCX7353 110 mg</th>
<th>BCX7353 150 mg</th>
<th>Placebo</th>
</tr>
</thead>
<tbody>
<tr>
<td>N = 158</td>
<td>N = 184</td>
<td>N = 39</td>
<td></td>
</tr>
</tbody>
</table>

### Subject Incidence of SAEs or Discontinuations due to AEs

<table>
<thead>
<tr>
<th>AEs Leading to Discontinuation of Study Drug</th>
<th>BCX7353 110 mg</th>
<th>BCX7353 150 mg</th>
<th>Placebo</th>
</tr>
</thead>
<tbody>
<tr>
<td>Drug-Related Serious AEs</td>
<td>2 (1.3%)</td>
<td>1 (0.5%)</td>
<td>0</td>
</tr>
<tr>
<td>Abdominal GI AEs</td>
<td>4 (2.5%)</td>
<td>7 (3.8%)</td>
<td>0</td>
</tr>
<tr>
<td>Abnormal Liver Function Test</td>
<td>3 (1.9%)</td>
<td>6 (3.3%)</td>
<td>0</td>
</tr>
<tr>
<td>Other AEs</td>
<td>4 (2.5%)</td>
<td>5 (2.7%)</td>
<td>1 (2.6%)</td>
</tr>
</tbody>
</table>

### Subject Incidence of Most Common GI Abdominal AEs Reported as Drug-Related

<table>
<thead>
<tr>
<th>Gastrointestinal Disorders System Organ Class</th>
<th>BCX7353 110 mg</th>
<th>BCX7353 150 mg</th>
<th>Placebo</th>
</tr>
</thead>
<tbody>
<tr>
<td>Nausea</td>
<td>10 (6.3%)</td>
<td>15 (8.2%)</td>
<td>6 (15.4%)</td>
</tr>
<tr>
<td>Abdominal pain</td>
<td>14 (8.9%)</td>
<td>16 (8.7%)</td>
<td>0</td>
</tr>
<tr>
<td>Diarrhea</td>
<td>10 (6.3%)</td>
<td>15 (8.2%)</td>
<td>0</td>
</tr>
<tr>
<td>Flatulence</td>
<td>4 (2.5%)</td>
<td>11 (6.0%)</td>
<td>1 (2.6%)</td>
</tr>
<tr>
<td>Abdominal pain upper</td>
<td>9 (5.7%)</td>
<td>7 (3.8%)</td>
<td>1 (2.6%)</td>
</tr>
<tr>
<td>Dyspepsia</td>
<td>8 (5.1%)</td>
<td>10 (5.4%)</td>
<td>2 (5.1%)</td>
</tr>
<tr>
<td>Abdominal discomfort</td>
<td>7 (4.4%)</td>
<td>6 (3.3%)</td>
<td>2 (5.1%)</td>
</tr>
<tr>
<td>Abdominal distension</td>
<td>5 (3.2%)</td>
<td>8 (4.3%)</td>
<td>2 (5.1%)</td>
</tr>
<tr>
<td>Vomiting</td>
<td>4 (2.5%)</td>
<td>7 (3.8%)</td>
<td>0</td>
</tr>
</tbody>
</table>

1: Gastroenteritis and hepatic enzyme increased in the same subject, events resolved after stopping study drug (ApeX-S)
2: Abdominal pain, event resolved after interrupting study drug (ApeX-S)
3: LFT abnormal, event resolved after stopping study drug (ApeX-S)
4: GI abdominal-related AEs were any AEs with a PT within the MedDRA 19.1 hierarchy under the high level group terms of GI signs and symptoms or GI motility and defecation conditions
5: One subject in this category had an infection and abnormal LFTs and is also counted in that row
6: For GI abdominal AEs occurring with a rate of at least 3% of BCX7353-treated subjects
<table>
<thead>
<tr>
<th>Market Sizing</th>
<th>US HAE Patients</th>
<th>US Physicians</th>
<th>US Payors</th>
</tr>
</thead>
<tbody>
<tr>
<td>• US prevalence study using administrative claims data</td>
<td>• 100 quantitative, 25-minute online surveys</td>
<td>• 175 quantitative, 20-minute online surveys</td>
<td>• 16 interviews with medical and pharmacy directors from insurance plans and PBMs covering &gt;100 million lives</td>
</tr>
<tr>
<td></td>
<td>• 26 individual, 60- to 75-minute qualitative interviews</td>
<td>• 43 individual, 60- to 75-minute qualitative interviews</td>
<td></td>
</tr>
</tbody>
</table>

Administrative Claims Analysis Estimates US HAE Population at ~10,000 Patients with ~7,500 Diagnosed & Treated

**Data Source:** Administrative claims from Symphony Integrated Dataverse (IDV) from 2017-2019 for >270 million US patients

**HAE Patient cohorts**
1. Diagnosed and treated with HAE-specific medication
2. Diagnosed but not treated with HAE-specific medication
3. Treated with HAE-specific medication but not diagnosed

**Claims Variables**
- Recurring claims with HAE ICD-9/10 diagnosis codes
- Complement function and/or level tests
- Recurring claims for HAE-specific medications

**National projections***
1. ~7,500 patients diagnosed and treated
2. ~1,700 patients diagnosed but not treated
3. ~600 patients treated but not diagnosed

Large, Quantitative Market Research Studies with US Patients and HAE-treating Physicians in July 2019 with 24-week APeX-2 Profile

100 HAE Patients

- 25-minute online survey
- Age 18+, diagnosed with Type I or II HAE
- Currently treating HAE or not currently treating and has 1+ attack every 3 months
- 50% recruited from HAEA patient organization
- 50% recruited via social media and online panels

175 HAE-Treating Physicians

- 20-minute online survey
- Allergist/Immunologist (n=100)
- Other specialty (n=75)
- Actively treats 2+ Type I or II HAE patients per year
- Study average = 7.6 patients/year
- Recruited via email and online panels

Physicians in this study treat 1,300 HAE patients representing over 10% of US HAE patients

Respondents Viewed a Blinded Profile of BCX7353 Based on 24-week Results from APeX-2

<table>
<thead>
<tr>
<th>Indication</th>
<th>Prophylactic treatment of HAE for patients 12 years and above</th>
</tr>
</thead>
<tbody>
<tr>
<td>Dosage</td>
<td>Take 1 capsule by mouth once per day</td>
</tr>
<tr>
<td>Clinical trial design</td>
<td>Patients who were experiencing an average of 3 HAE attacks per month took Treatment X or a placebo (an inactive drug often used in clinical trials) for 6 months</td>
</tr>
<tr>
<td>Efficacy</td>
<td>Patients taking Treatment X had 44% fewer HAE attacks overall than patients taking a placebo during the 6-month clinical trial</td>
</tr>
<tr>
<td></td>
<td>Half (50%) of patients taking Treatment X reduced their number of HAE attacks by 70% or more between the beginning and end of the trial</td>
</tr>
<tr>
<td></td>
<td>About 1 in 4 patients (23%) taking Treatment X reduced their number of HAE attacks by 90% or more between beginning and end of the trial</td>
</tr>
<tr>
<td>Safety and tolerability</td>
<td>Adverse events from Treatment X were generally mild and similar to placebo</td>
</tr>
<tr>
<td></td>
<td>The most common side effects experienced more often with Treatment X were short episodes of mild diarrhea or vomiting experienced by about 10% of patients</td>
</tr>
</tbody>
</table>

Strong HAE Patient Demand for BCX7353:

59% of Patients Expressed High Willingness to use BCX7353
Rises to 71% with Physician Recommendation

Patient Willingness to Use BCX7353 (N=100)

- Not Willing (rated 0-2): 5%
- Somewhat Willing (rated 3-7): 22%
- Very Willing (rated 8-10): 59%
- Unsure: 14%

Patient Willingness to Use BCX7353 with MD Recommendation (N=100)

- Not Willing (rated 0-2): 5%
- Somewhat Willing (rated 3-7): 21%
- Very Willing (rated 8-10): 71%
- Unsure: 3%

All Qualified HAE Patients (n=100)
Rated on a scale where a “0” indicates “Not at all willing”, and a “10” indicates “Extremely willing”

Source: BioCryst Proprietary Market Research Study, 2019. Respondents were shown a blinded profile (Treatment X) of BCX7353, 24-week results of APeX-2
Prophylaxis Patients are Very Willing to Use BCX7353—even Those Very Satisfied with their Current Injectable Prophylactic Treatment

<table>
<thead>
<tr>
<th>Treatment</th>
<th>% of Those Very Satisfied on Current Therapy</th>
<th>% of Total</th>
</tr>
</thead>
<tbody>
<tr>
<td>Cinryze (N=24)</td>
<td>63%</td>
<td>15 of 24</td>
</tr>
<tr>
<td></td>
<td>50%</td>
<td>5 of 10</td>
</tr>
<tr>
<td>Haegarda (N=25)</td>
<td>60%</td>
<td>15 of 25</td>
</tr>
<tr>
<td></td>
<td>50%</td>
<td>8 of 16</td>
</tr>
<tr>
<td>Takhzyro (N=30)</td>
<td>47%</td>
<td>14 of 30</td>
</tr>
<tr>
<td></td>
<td>48%</td>
<td>13 of 27</td>
</tr>
</tbody>
</table>

All Current Prophylaxis Users—“Very Willing” & “Very Satisfied” = Top 3 Box (rated 8,9,10 on 10 point scale)

Willingness rated on a scale where a “0” indicates “Not at all willing”, and a “10” indicates “Extremely willing”

Satisfaction with current treatment rated on a scale where a “0” indicates “Not at all satisfied”, and a “10” indicates “Extremely satisfied”

Source: BioCryst Proprietary Market Research Study, 2019. Respondents were shown a blinded profile (Treatment X) of BCX7353, 24-week results of APeX-2
Patients Report Breakthrough Attacks with Injectable/Infused Treatments

Mean Patient-Reported Attacks in Past 3 Months by Prophylaxis Medication

Cinryze (n=24) - 1.6
Haegarda (n=25) - 0.9
Takhzyro (n=30) - 1.8

Currently Taking Medication Prophylactically

Physicians Expect to Prescribe BCX7353 for Over 40% of HAE Patients
80% of HAE Patients Expected to be on Some Form of Prophylaxis

**Patient Allocation by Medication Today and in Future with BCX7353 Available – Mean % of Patients**

- **Current Treatment Allocation**
  - Not sure: 4%
  - No medication: 25%
  - Acute only: 10%
  - Acute - taken as prophy: 58%

- **Future Treatment Allocation**
  - Not sure: 5%
  - No medication: 8%
  - Acute only: 6%
  - Acute - taken as prophy: 39%
  - Current Prophylaxis: 41%

*All Qualified Respondents (n=175)*

Source: BioCryst Proprietary Market Research Study, 2019. Respondents were shown a blinded profile (Treatment X) of BCX7353, 24-week results of APeX-2. Physicians were asked to perform a patient allocation.
### Clinical Trial Experience Consistent with Market Research—
Patients on Injectable Prophylaxis Switch to Oral Berotralstat

| Physicians’ expectations in market research | ~50% of future use of berotralstat will come from patients switching from other prophylaxis treatments |
| APeX-2 enrollment | 44% of patients treated previously with injected or infused C1 inhibitor prophylaxis |
| APeX-S enrollment in the United States | ~50% of patients enrolled since mid-2019 previously treated with Takhzyro, Haegarda or Cinryze prophylaxis |

Sources: BioCryst proprietary qualitative research, 2019; APeX-2 and APeX-S trial enrollment
# Insights from Long-term Patients in APeX-2: Why they Stay on Oral, Once-Daily Berotralstat

| Efficacy | “In the past 3 months I may have had to fall back on rescue maybe 3 times, which is fantastic. I’ll take that all day long. Three times in 3 months compared to twice a week [on Haegarda], this is so much better.” |
|          | “If I felt like a swelling going on in my stomach. Being on [berotralstat] never allowed that swelling to really run its course. I was able to eat and sleep and exercise normally… [without berotralstat] I would have had to hit pause for about 3 days.” |
|          | “I started to feel like I was having less HAE attacks, but more importantly, they were less severe and would be very easily controlled with the acute medications that I took.” |
| Tolerability | “I haven’t really experienced any side effects. Early on it sort of wanted to bother my stomach, but not anymore because now I know [to take it with a meal].” |
| Less burden and improved quality of life | “So much freer not to have all [that medicine] in your refrigerator, in your purse, when you travel... So much easier as far as not having to schedule time to mix drug and infuse it.” |
|          | “I travel a lot for work...[berotralstat] gave me an opportunity to never miss a treatment. It was critical in doing that. If I’d had to carry around a needle or a shot it would have been a very different process to have managed.” |
|          | “After several years of being a pincushion it was nice to be able to take a pill” |
|          | “It was just exciting to see the difference the medication was making... All my hopes and dreams for what I was praying for started to come true, everything started to happen the way I was hoping.” |
|          | “You don’t even realize how hard [treating HAE] is on you right now, ’cause this is all you’ve ever known. So I can’t wait. As soon as this gets FDA approved... I’m on a bunch of patient education groups for HAE, and I’ve had to stay quiet about how good this works.” |

US Payors Anticipate Providing Coverage for Berotralstat

Medical & Pharmacy Directors
- 80M covered lives
- Positive reaction to therapeutic value of profile
- Accept that treatment approach is individualized choice made by patients and HCPs

PBM
- 110M covered lives
- Willing to cover new therapies in HAE
- Broad willingness to pay for BCX7353 if priced in line with the market basket of prophylactic treatments

Source: BioCryst Proprietary Research, 2019. Sample included 5 national insurance plans, 7 regional plans, 2 IDNs, and 2 national PBMs. Respondents were shown a blinded profile (Treatment X) of BCX7353, 24-week results of APeX-2.
Berotralstat for HAE Prophylaxis: Data Supports Global Peak Market Opportunity >$500M

<table>
<thead>
<tr>
<th>Clinical Data</th>
<th>Prevalence</th>
<th>Treatment Paradigm</th>
</tr>
</thead>
<tbody>
<tr>
<td>Consistent, clinically meaningful benefit demonstrated through 48 weeks</td>
<td>~10,000 (US) HAE Patients</td>
<td>Physicians expect shift to ~80% prophylaxis</td>
</tr>
<tr>
<td>Safe and generally well-tolerated</td>
<td>~7,500 diagnosed and treated</td>
<td></td>
</tr>
</tbody>
</table>

**Strong Demand for Berotralstat Product Profile and Benefit**
- Overall, 60-70% of patients very willing to use
- Physicians intending to prescribe to >40% of patients
- Payors acknowledge therapeutic value and broad willingness to pay
Preparing for a Successful Commercial Launch

- Building out critical launch elements based on our detailed market understanding:
  - Marketing strategy, messages and tactics
  - Sales force structure and targeting
  - Market access strategies
  - Developing a best-in-class patient services and hub program

- Medical Affairs team deployed and engaging with the KOL community

- Experienced U.S. and EU commercial leadership team in place

- Robust dual-source supply chain to support commercial launch
Multiple Potential Global Approvals in 2020-2021

- **Orphan Drug 2017**
  - NDA accepted
  - PDUFA Date: 12/3/20

- **Sakigake 2015**
  - JNDA accepted
  - Approval 2H 2020

- **Orphan Drug 2018**
  - MAA accepted Mar 2020
  - CHMP Opinion: ~12 mo

2020

2020

2021
APeX-J – Primary Efficacy Endpoint was Met for Berotralstat 150 mg
Total Enrollment: 19 (7 at 150 mg, 6 at 110mg, 6 placebo)

<table>
<thead>
<tr>
<th>Arm</th>
<th>N</th>
<th>Rate</th>
<th>Attack rate ratio active/placebo (95% CI)</th>
<th>Percent reduction from placebo (95% CI)</th>
<th>P value</th>
</tr>
</thead>
<tbody>
<tr>
<td>Berotralstat 150 mg</td>
<td>7</td>
<td>1.11</td>
<td>0.51 (0.33, 0.80)</td>
<td>49.1 (20.4, 67.5)</td>
<td>0.003</td>
</tr>
<tr>
<td>Berotralstat 110 mg</td>
<td>6</td>
<td>1.64</td>
<td>0.75 (0.50, 1.14)</td>
<td>24.6 (-14.0, 50.1)</td>
<td>0.181</td>
</tr>
<tr>
<td>Placebo</td>
<td>6</td>
<td>2.18</td>
<td>-</td>
<td>-</td>
<td>-</td>
</tr>
</tbody>
</table>

* Statistical analysis is based on a negative binomial regression model. The number of expert-confirmed events is included as the dependent variable, the treatment is included as a fixed effect, baseline expert-confirmed event rate is included as a covariate, and the logarithm of duration on treatment is included as an offset variable.
Berotralstat 150 mg Shows Consistent, Sustained Reduction in Attacks Over 24 Weeks
## Overall Safety Summary:
Berotralstat was Safe and Generally Well Tolerated

<table>
<thead>
<tr>
<th>Treatment-emergent (TE) Adverse Events (AEs) or Discontinuations (DCs) due to TEAEs</th>
<th>Berotralstat 110 mg</th>
<th>Berotralstat 150 mg</th>
<th>Placebo</th>
</tr>
</thead>
<tbody>
<tr>
<td></td>
<td>N = 6</td>
<td>N = 7</td>
<td>N = 6</td>
</tr>
<tr>
<td>Any Drug-Related TEAEs</td>
<td>2 (33.3%)</td>
<td>2 (28.6%)</td>
<td>2 (33.3%)</td>
</tr>
<tr>
<td>Drug-Related Serious TEAEs</td>
<td>0</td>
<td>0</td>
<td>0</td>
</tr>
<tr>
<td>Drug-Related Grade 3 or 4 TEAEs</td>
<td>0</td>
<td>0</td>
<td>0</td>
</tr>
<tr>
<td>Any Drug-Related Abdominal GI TEAE</td>
<td>2 (33.3%)</td>
<td>1 (14.3%)</td>
<td>1 (16.7%)</td>
</tr>
<tr>
<td>Most Common¹ Drug-Related TEAEs</td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Abdominal discomfort</td>
<td>1 (16.7%)</td>
<td>0</td>
<td>1 (16.7%)</td>
</tr>
<tr>
<td>DCs due to TEAEs</td>
<td>0</td>
<td>0</td>
<td>1 (16.7%)²</td>
</tr>
</tbody>
</table>

¹ Occurring in >1 subject
² One placebo subject discontinued due to urticaria
Unique Market Opportunity in Japan

Japanese Market Growth Potential

- Berotralstat would be 1st approved prophylactic HAE therapy in Japan
- Active KOL base of treating physicians with strong interest in new therapies for patients
- Lower awareness of disease and lack of standard-of-care treatments have limited diagnosis rates compared to US
- Very active patient advocacy groups increasing awareness in HAE prophylaxis

Berotralstat for HAE Prophylaxis: Japanese Partnership with Torii
*Non-dilutive Capital + Access to Unique Market with Large Unmet Need*

- $42 million in upfront and milestones
  - $22 million upfront
  - Up to $20 million with approval + threshold pricing
  - Royalties from mid-teens up to potentially 40%
- Proven, committed partner
- JNDA submitted Q1 2020
- Sakigake designation could enable Japan to be 1st global approval (2H 2020)
Factor D: Outstanding Target for Complement-mediated Diseases

Factor D is an ideal target:

- Required for the alternative pathway (AP) to work
- Target is the same in PNH, nephritis, and other AP diseases
- Circulating Factor D levels are the lowest of any complement pathway enzyme
- Levels do not increase with inflammatory illnesses
- Unique enzyme structure enables design of inhibitors with better specificity against other serine proteases

Application to BCX9930 Development:

- Doses of BCX9930 that block Factor D will inhibit the AP independent of the disease setting
- Proof of concept in PNH provides POC for other diseases of the alternative pathway
- Less drug required for inhibition compared to other complement targets
- No dose adjustment when patients get illnesses like influenza
- Can lead to a better safety margin
Targeting Overactive Alternative Pathway Could Treat Many Complement-mediated Diseases

Pathology of Renal Diseases Associated with Dysfunction of the Alternative Pathway of Complement: C3 Glomerulopathy and Atypical Hemolytic Uremic Syndrome (aHUS)

Sanjeev Sethi, MD, PhD1  Fernando C. Fervenza, MD, PhD2

CJASN  Clinical Journal of American Society of Nephrology

Causes of Alternative Pathway Dysregulation in Dense Deposit Disease


Anti-complement Treatment for Paroxysmal Nocturnal Hemoglobinuria: Time for Proximal Complement Inhibition? A Position Paper From the SAAWP of the EBMT

Antonio M. Risitano1,2,*, Serena Marotta1,2,*, Patrizia Ricci1,*, Luana Marano1,*, Camilla Frieri1,*, Fabiana Cacace1,*, Michele Sica1,*, Austin Kulasekaranraj1,2,*, Rodrigo T. Calado5,*, Phillip Scheinberg5,*, Rosario Notaro5,6 and Regis Peffault de Latour5,7 on behalf of the Severe Aplastic Anemia Working Party of the European group for Bone Marrow Transplantation
# BCX9930 28-day PNH Proof of Concept Study Design

## Key Outcome Measures
- LDH, hemoglobin
- Safety
- PK
- PD

<table>
<thead>
<tr>
<th>Total of 28 days of BCX9930 dosing</th>
</tr>
</thead>
<tbody>
<tr>
<td>Period 1 days 1-14</td>
</tr>
<tr>
<td>Period 2 days 15-28</td>
</tr>
</tbody>
</table>

### Subjects with PNH who are naïve to C5-INH treatments: BCX9930 monotherapy

<table>
<thead>
<tr>
<th>Cohort 1: n = up to 4*</th>
<th>50 mg BID days 1-14</th>
<th>100 mg BID days 15-28</th>
</tr>
</thead>
<tbody>
<tr>
<td>Cohort 2: n = up to 4</td>
<td>200 mg BID days 1-14</td>
<td>400 mg BID days 15-28</td>
</tr>
</tbody>
</table>

### Subjects with PNH with poor response to C5-INH: BCX9930 plus continued C5-INH

<table>
<thead>
<tr>
<th>Cohort 1: n = up to 4</th>
<th>50 mg BID days 1-14</th>
<th>100 mg BID days 15-28</th>
</tr>
</thead>
<tbody>
<tr>
<td>Cohort 2: n = up to 4</td>
<td>200 mg BID days 1-14</td>
<td>400 mg BID days 15-28</td>
</tr>
</tbody>
</table>

*Cohort 1 enrolment for subjects naïve to C5-INH is completed*
## Treatment-naïve PNH Patients had Severe Disease

<table>
<thead>
<tr>
<th>Pre-treatment Characteristics</th>
<th>Subject 1</th>
<th>Subject 2</th>
<th>Subject 3</th>
</tr>
</thead>
<tbody>
<tr>
<td>PNH duration, years</td>
<td>8</td>
<td>4</td>
<td>3</td>
</tr>
<tr>
<td>History of aplastic anemia</td>
<td>no</td>
<td>no</td>
<td>yes</td>
</tr>
<tr>
<td>History of thrombosis</td>
<td>yes</td>
<td>no</td>
<td>no</td>
</tr>
<tr>
<td>LDH, IU/L</td>
<td>2205</td>
<td>2497</td>
<td>835</td>
</tr>
<tr>
<td><em>LDH</em> × <em>ULN</em></td>
<td>9.8</td>
<td>11.0</td>
<td>3.7</td>
</tr>
<tr>
<td>Hemoglobin, g/dL</td>
<td>8.2</td>
<td>7.0</td>
<td>6.0</td>
</tr>
<tr>
<td>Reticulocytes, 10^3 cells/µL</td>
<td>220</td>
<td>285</td>
<td>130</td>
</tr>
<tr>
<td>Total bilirubin, mg/dL</td>
<td>3.33</td>
<td>1.47</td>
<td>1.12</td>
</tr>
<tr>
<td>PNH type III erythrocyte clone size, %</td>
<td>89</td>
<td>41</td>
<td>49</td>
</tr>
<tr>
<td>Units of RBC transfused in 52 weeks prior to screening</td>
<td>0</td>
<td>13</td>
<td>0</td>
</tr>
<tr>
<td>Units of RBC transfused in 12 weeks prior to screening</td>
<td>0</td>
<td>2</td>
<td>0</td>
</tr>
</tbody>
</table>

*Laboratory values for LDH, reticulocyte count, total bilirubin and PNH type III erythrocyte clone size are average of available screening and baseline results. Hemoglobin is last available value prior to Day 1 of BCX9930 administration. Study is ongoing – preliminary data.*
Dose-dependent Improvement Across Key Indicators in Treatment-naïve PNH Subjects Receiving BCX9930 Monotherapy

Study is ongoing – preliminary data. Assays pending for RBC clone size on day 28. Asterisk indicates RBC transfusion in Subject 2 on day 15.
BCX9930 Data Provides Strong Support for Oral Monotherapy in PNH

Safety & Tolerability in PNH, n=3

- BCX9930 has been safe and generally well-tolerated in cohort 1 at low doses of 50 mg bid days 1-14 followed by 100 mg bid days 15-28
- No BCX9930-related serious adverse events
- No safety signals in routine monitoring of:
  - Vital signs, ECGs, or laboratory evaluations of hematology, coagulation, urinalysis, or clinical chemistry
- One death unrelated to study drug following 28 day study period
- 3/3 subjects had moderate headache resolving in <1-3 days soon after starting study drug
- No rash observed

Activity in PNH at low doses, n=3

- Prompt and sustained reductions in LDH (3/3) and reticulocytes (2/3)
- Increasing PNH clone size and Hb
- Investigator assessed clinical benefit in 3/3 patients, all continued to long-term extension

Next steps

- Open 200/400 mg bid cohort for C5-inhibitor naïve patients after completing cohort 1, data expected Q3 2020
- Enroll C5-inhibitor poor responders in 200/400 mg bid cohort in Q3 2020, data expected by YE 2020
Single Dose PK Profile of Oral BCX9930 in Healthy Subjects

**PK Profile of Single Dose BCX9930**

- **ng/mL, mean (SEM)**
  - Dose levels: 100 mg, 300 mg, 600 mg, 1200 mg
  - Log-log slope: 1.02

**Dose-Proportional Exposure of Single Dose BCX9930**

- **C<sub>max</sub>, ng/mL**
  - Mean (SD) C<sub>max</sub>
  - Regression (95% CI band)
  - Log-log slope: 1.02

- **AUC<sub>0-24h</sub>, ng.h/mL**
  - Mean (SD) AUC<sub>0-24h</sub>
  - Regression (95% CI band)
  - Log-log slope: 1.04

**Hours post-dose:** 0, 4, 8, 12, 16, 20, 24
Suppression of AP Activity After Single Oral Doses of BCX9930

Alternative pathway complement activity in healthy subjects: oral BCX9930 single dose

**Assay: AP Wieslab**

- 300 mg
- 100 mg
- 600 mg
- 1200 mg

**Assay: AP Hemolysis**

- 100 mg
- 300 mg
- 600 mg
- 1200 mg

Hours post-dose

AP Wieslab, % of predose, mean (SEM)

AP Hemolysis, % of predose, mean (SEM)
Clear Dose-response in AP Inhibition – Consistent, Sustained Suppression at 200/400 mg Q12h

Last dose of BCX9930 on Q12 hr schedule was administered at time 0, and results through 24 hours post-dose are shown.
Greater Exposure at 200/400 mg with >98% Sustained Alternative Pathway Suppression in Both Assays

BCX9930 PK & PD Profile at Steady State

BCX9930 PD Profile: Day 1 and Steady State PK Troughs
Successful BCX9930 SAD/MAD Supports Monotherapy for Diseases of the Alternative Pathway

Safety & Tolerability: Healthy Subjects

- Study drug was safe and generally well-tolerated at all doses
- No serious adverse events
- No safety signals in routine monitoring of:
  - Vital signs, ECGs, or laboratory evaluations of hematology, coagulation, urinalysis, or clinical chemistry
- Benign rash in majority of MAD subjects was self-limited and resolved within a median of 5 days of onset
- No dose-related safety signals observed

PK/PD in Healthy Subjects

- Linear, dose-proportional exposure
- Dose-related suppression of AP of complement functional activity
- > 98% inhibition of AP in both AP Wieslab and AP hemolysis assays at steady-state dosing for doses of 200 mg Q12h and 400 mg Q12h

Next Steps

- Test supratherapeutic doses to finish SAD/MAD
- Explore once-daily dosing
Galidesivir Clinical Trial in COVID-19 Enrolling Patients

Part 1 (n=24)

- Cohort 1: GVR n=6, PBO n=2
  - 10 mg/kg then 2 mg/kg q12h×13
- Cohort 2: GVR n=6, PBO n=2
  - 10 mg/kg then 5 mg/kg q12h×13
- Cohort 3: GVR n=6, PBO n=2
  - 20 mg/kg then 5 mg/kg q12h×13

Part 2 - Randomized 2:1 (GVR:PBO)

- Cohort: Dose selected from Part 1 (n=42)

Key Outcome Measures

- Safety
- PK
- Viral Load Reduction
- Changes in clinical signs and symptoms
# Cash position & 2020 guidance (in millions)

<table>
<thead>
<tr>
<th>Description</th>
<th>Amount</th>
</tr>
</thead>
<tbody>
<tr>
<td>Cash &amp; investments at December 31, 2019</td>
<td>$138</td>
</tr>
<tr>
<td>Cash &amp; investments at March 31, 2020</td>
<td>$115</td>
</tr>
<tr>
<td>Proforma - Cash &amp; investments at March 31, 2020 A</td>
<td>$222</td>
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<tr>
<td>Senior Credit Facility</td>
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## FY 2020 GUIDANCE

<table>
<thead>
<tr>
<th>Description</th>
<th>Range</th>
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</thead>
<tbody>
<tr>
<td>Net operating cash utilization</td>
<td>$125 – 150</td>
</tr>
<tr>
<td>Operating expenses B</td>
<td>$135 – 160</td>
</tr>
</tbody>
</table>

A - Includes net proceeds from Q2 public equity offering.

B - Excludes equity-based compensation.