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 http://ir.biocryst.com/financial-information/sec-filings
Delivering extraordinary
Empowering ordinary

BioCryst develops novel oral medicines for rare disease to help patients experience a normal quality of life.
The Year Ahead: Approvals, Launches and Data

2019 Accomplishments

- Submitted NDA to FDA for berotralstat
- Initiated oral Factor D Phase 1 trial for complement-mediated diseases
- Initiated oral ALK2 inhibitor Phase 1 study for development in FOP
- Added ~$100M in capital during 4Q 2019

2020 Priorities

1. Obtain berotralstat approvals in U.S. + Japan and submit MAA to EMA
2. Prepare commercial infrastructure for successful launches in the US & EU (+ support Torii in Japan)
3. Achieve proof of concept for oral Factor-D inhibitor in PNH patients
4. Continue advancing rare disease portfolio via in-house R&D or out-licensing partnerships
Berotralstat (BCX7353):
Oral, Once Daily to Prevent HAE Attacks
**APeX-2 Study – 48-week Analysis**

- Eligible HAE subjects
- Run-in period of 14-56 days to qualify
- No androgens for 28 days before screening

**Subjects Enrolled and Follow-up**

<table>
<thead>
<tr>
<th>Subjects Enrolled and Follow-up</th>
<th>BCX7353 110 mg</th>
<th>BCX7353 150 mg</th>
<th>Placebo</th>
</tr>
</thead>
<tbody>
<tr>
<td>Subjects enrolled [ITT Population]</td>
<td>41</td>
<td>40</td>
<td>40</td>
</tr>
<tr>
<td>Subjects enrolled and dosed [Safety Population]</td>
<td>41</td>
<td>40</td>
<td>39</td>
</tr>
<tr>
<td>Subjects who completed 24 weeks of study drug (Part 1)</td>
<td>37</td>
<td>37</td>
<td>34</td>
</tr>
<tr>
<td>Randomization to active drug at conclusion of Part 1 for placebo subjects</td>
<td>-</td>
<td>-</td>
<td>110 mg 150 mg</td>
</tr>
<tr>
<td>Placebo subjects who were randomized (110 mg:150 mg) at 24 weeks</td>
<td>-</td>
<td>-</td>
<td>17      17</td>
</tr>
<tr>
<td>Subjects continuing on study, not yet reaching 48 weeks of study drug</td>
<td>3</td>
<td>1</td>
<td>1       1</td>
</tr>
<tr>
<td>Subjects who discontinued study drug between 24 and 48 weeks</td>
<td>9</td>
<td>6</td>
<td>2       3</td>
</tr>
<tr>
<td>Subjects who completed 48 weeks of study drug* [Completers Population]</td>
<td>25 (61%)</td>
<td>30 (75%)</td>
<td>14 (82%) 13 (76%)</td>
</tr>
<tr>
<td>Weeks of BCX7353 treatment for Completers Population</td>
<td>48</td>
<td>48</td>
<td>24      24</td>
</tr>
</tbody>
</table>

**Previous Prophylactic Treatments for HAE**

<table>
<thead>
<tr>
<th>Previous Prophylactic Treatments for HAE</th>
<th>BCX7353 110 mg</th>
<th>BCX7353 150 mg</th>
<th>Placebo</th>
</tr>
</thead>
<tbody>
<tr>
<td>Androgens</td>
<td>19 (46%)</td>
<td>21 (53%)</td>
<td>25 (63%)</td>
</tr>
<tr>
<td>C1-INH</td>
<td>16 (39%)</td>
<td>21 (53%)</td>
<td>16 (40%)</td>
</tr>
</tbody>
</table>

* Study drug includes BCX7353 through the 48-week visit or placebo through the 24-week visit followed by BCX7353 through the 48-week visit (i.e., for 24 weeks).
APeX-S Study – 48-week Analysis

- Eligible HAE subjects
- No run-in period
- No androgens for 7 days before entry

Allocated to dose

- Daily oral BCX7353
- Regular clinic visits
- Safety assessments
- HAE attacks recorded

Follow-up

48-week analysis

<table>
<thead>
<tr>
<th>Subjects Enrolled and Follow-up</th>
<th>BCX7353 110 mg</th>
<th>BCX7353 150 mg</th>
</tr>
</thead>
<tbody>
<tr>
<td>Subjects enrolled [Safety Population]</td>
<td>100</td>
<td>127</td>
</tr>
<tr>
<td>Subjects continuing on study, not yet reaching 48 weeks of study drug</td>
<td>44</td>
<td>23</td>
</tr>
<tr>
<td>Subjects who discontinued study drug before 48 weeks</td>
<td>26</td>
<td>31</td>
</tr>
<tr>
<td>Subjects who completed 48 weeks of BCX7353 [Completers Population]</td>
<td>30</td>
<td>73</td>
</tr>
</tbody>
</table>

<table>
<thead>
<tr>
<th>Past Prophylactic Treatment of HAE</th>
<th>BCX7353 110 mg</th>
<th>BCX7353 150 mg</th>
</tr>
</thead>
<tbody>
<tr>
<td>Androgens</td>
<td>69 (69%)</td>
<td>84 (66%)</td>
</tr>
<tr>
<td>C1-INH</td>
<td>22 (22%)</td>
<td>32 (25%)</td>
</tr>
</tbody>
</table>
Rapid and Sustained HAE Attack Rate Reduction: Analyses for APeX-2: 150 mg and Placebo 48-week Completers

![Graph showing attack rate per 4-week month for BCX7353 150 mg QD and Placebo](image)

**APeX-2 Attack Rate by Month, Completers Analysis**
- BCX7353 150 mg QD
- Placebo and Placebo-to-Active

**Month of study drug**
- BL
- 3
- 6
- 9
- 12

**Attacks rate per 4-week month, mean (SEM)**
- BCX7353 150 mg, n=30
- 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

Month of study drug

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

APeX-2 Completers Analysis, 150 mg QD BCX7353

APeX-S Completers Analysis, 150 mg QD BCX7353
# Safety and Tolerability Confirmed in Integrated 48-week Analysis

## Integrated Safety Summary – APeX-2 and APeX-S

<table>
<thead>
<tr>
<th>Subjects enrolled and dosed [Safety Population]</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

### Drug-Related Serious AEs
- 2 (1.3%) 1, 2
- 1 (0.5%) 3
- 0

### AEs Leading to Discontinuation of Study Drug

<table>
<thead>
<tr>
<th>Abdominal GI AEs 4</th>
<th>4 (2.5%)</th>
<th>7 (3.8%)</th>
<th>0</th>
</tr>
</thead>
<tbody>
<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%) 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 6

<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
Berotralstat (BCX7353): Market Research Update
Robust Market Research Since APeX-2

<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

<table>
<thead>
<tr>
<th>100 HAE Patients</th>
<th>175 HAE-Treating Physicians</th>
</tr>
</thead>
<tbody>
<tr>
<td>• 25-minute online survey</td>
<td>• 20-minute online survey</td>
</tr>
<tr>
<td>• Age 18+, diagnosed with Type I or II HAE</td>
<td>• Allergist/Immunologist (n=100)</td>
</tr>
<tr>
<td>• Currently treating HAE or not currently treating and has 1+ attack every 3 months</td>
<td>• Other specialty (n=75)</td>
</tr>
<tr>
<td>• 50% recruited from HAEA patient organization</td>
<td>• Actively treats 2+ Type I or II HAE patients per year</td>
</tr>
<tr>
<td>• 50% recruited via social media and online panels</td>
<td>• Study average = 7.6 patients/year</td>
</tr>
<tr>
<td></td>
<td>• Recruited via email and online panels</td>
</tr>
</tbody>
</table>

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><strong>Indication</strong></th>
<th>Prophylactic treatment of HAE for patients 12 years and above</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>Dosage</strong></td>
<td>Take 1 capsule by mouth once per day</td>
</tr>
<tr>
<td><strong>Clinical trial design</strong></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><strong>Efficacy</strong></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><strong>Safety and tolerability</strong></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)

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

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

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

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>Prophylaxis Patients VERY WILLING to Use BCX7353</th>
</tr>
</thead>
<tbody>
<tr>
<td>Cinryze (N=24)</td>
</tr>
<tr>
<td></td>
</tr>
<tr>
<td>Haegarda (N=25)</td>
</tr>
<tr>
<td></td>
</tr>
<tr>
<td>Takhzyro (N=30)</td>
</tr>
<tr>
<td></td>
</tr>
</tbody>
</table>

- % of those Very Satisfied on current therapy
- % of Total

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

Patients Are Coping With Their Injectable Therapies  
*They Want the Ease that Only Berotralstat can Offer*

<table>
<thead>
<tr>
<th>Theme</th>
<th>Quote</th>
</tr>
</thead>
<tbody>
<tr>
<td>Time saving</td>
<td>“Make HAE less a part of my life...less time consuming...getting on with my day. A lot of times I am so rushed and I don’t have the time to do simple things, like do laundry or go grocery shopping. Being able to do this quickly...this is one thing that will be a lot simpler and help with my overall day.”</td>
</tr>
<tr>
<td>Less to think about and coordinate</td>
<td>“You can focus on doing other things that are more important in life. I’d rather spend my time doing other things...going out with friends, spending time with my grandson.”</td>
</tr>
<tr>
<td>Less hassle—inconvenience</td>
<td>“Less of a hassle for me; I work full time, I have two kids and I have a stressful, difficult life...anything I can do to prevent attacks in an easier way is less of a burden on me. Injections can be burdensome, injection site reactions, pain and swelling, dizziness.”</td>
</tr>
<tr>
<td>Less burden</td>
<td>“With medications, you have to make sure that they’re kept in a fridge, you have to make sure that when you take them to travel, that they don’t get too hot, you have to bring ice packs, you have to bring coolers. The fact that you don’t have to do that with this, again, just makes it easier. You don’t have to worry about keeping it a certain temperature.”</td>
</tr>
<tr>
<td>Not painful</td>
<td>“Less painful, not that using needles is all that painful, but it would be less painful. Probably less work behind it. Even if it’s just drawing the medication out of a vial, it’s still some work that you have to do. I’d almost say that it’s safer because you’re not injecting something into your blood, or on your skin. You’re swallowing a pill.”</td>
</tr>
<tr>
<td>Better routine</td>
<td>“Most people have a morning routine, whether it’s vitamins or taking other medication...so it would be easier than remembering every two weeks. Not worrying about the shipment, keeping it refrigerated, bent needles, the prep, etc.”</td>
</tr>
</tbody>
</table>

Source: BioCryst proprietary qualitative research, 2019.
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: 4%
- Acute only: 25%
- Acute - taken as prophy: 10%
- Current Prophylaxis: 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.
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
- Willing to cover new therapies in HAE

PBM

110M covered lives

- 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.
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

- Deployed competitively-sized MSL team to call on top-tier HAE treaters

- Attracting commercial leadership with extensive record of success in rare disease

- Developing a best-in-class patient services and hub program
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
Multiple Potential Global Approvals in 2020-2021

- **Orphan Drug 2017**
  - NDA submitted December 2019
  - 2020

- **Sakigake 2015**
  - JNDA submission Q1 2020
  - 2020

- **Orphan Drug 2018**
  - MAA submission Q1 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>Screening / Run-in 8 Weeks</th>
<th>Part 1 24 Week Treatment</th>
</tr>
</thead>
<tbody>
<tr>
<td></td>
<td>BCX7353 150 mg QD*</td>
</tr>
<tr>
<td></td>
<td>BCX7353 110 mg QD*</td>
</tr>
<tr>
<td></td>
<td>Placebo QD</td>
</tr>
</tbody>
</table>

**Primary endpoint: expert-confirmed angioedema attacks, rate/month***

<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>N</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
- Sakigake designation could enable Japan to be 1st global approval
- JNDA on-track for Q1 2020
BCX9930
Oral Factor D Inhibitor for Complement-mediated Diseases
The only marketed complement inhibitors are IV-infused

2018 sales from 3 indications, 55% ex-US

Orphan indications, each with >$1B potential sales

* SOLIRIS® (eculizumab) is indicated for in paroxysmal nocturnal hemoglobinuria (PNH), atypical hemolytic uremic syndrome (aHUS), generalized myastenia gravis (gMG), and neuromyelitis optica (NMO) spectrum disorder; and ULTOMIRIS® (ravulizumab-cwvz) is indicated for PNH and aHUS; both products are manufactured by Alexion Pharmaceuticals, Inc.

** SOLIRIS® 2018 sales for PNH, aHUS, and gMG, reported 2/4/19

*** Additional current and potential orphan indications for complement inhibitors include, but are not limited to, NMO, ANCA-associated vasculitis (AAV), C3 glomerulonephritis (C3G), IgA nephropathy (IgAN), warm autoimmune hemolytic anemia (wAIHA), focal segmental glomerulosclerosis (FSGS), and cold agglutinin disease (CAD)
BCX9930 Phase 1 Trial Design & Progress

**Part 1 – Single ascending dose**
- Healthy subjects
- PK & PD
- Safety and tolerability
- 8 subjects per cohort
  - 6:2 active : placebo
- 6 dose levels
- Completed

**Part 2 – Multiple ascending dose**
- Healthy subjects
- PK & PD
- Safety and tolerability
- 12 subjects per cohort
  - 10:2 active : placebo
- Multiple dose levels
- Ongoing

**Part 3 – Proof of concept in PNH patients**
- Poor responders to eculizumab or ravulizumab, or naïve to treatment
- Up to 16 patients total
- Multiple dose levels

- Part 1: SAD completed with cohorts from 10 to 1200 mg
- Part 2: Three MAD cohorts completed
  - 50 mg Q12hr x 7 days and 100 mg Q12hr x 7 days with concomitant antibiotic
  - 50 mg Q12hr x 14 days with vaccination
- Part 3: PNH proof of concept data expected 1H 2020
Single Dose PK Profile of Oral BCX9930 in Healthy Subjects

**PK Profile of Single Dose BCX9930**

![Graph showing PK profile with different doses (100 mg, 300 mg, 600 mg, 1200 mg) and time points (0-24 hours).]

**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>0-24</sub> ng·h/mL**
  - Mean (SD) AUC<sub>0-24</sub>
  - Regression (95% CI band)
  - Log-log slope: 1.04
Suppression of AP Activity After Single Oral Doses of BCX9930

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

Assay: AP Hemolysis

Assay: AP Wieslab

AP Hemolysis, % of predose, mean (SEM)

Hours post-dose

0 4 8 12 16 20 24

100 mg
300 mg
600 mg
1200 mg

AP Wieslab, % of predose, mean (SEM)

Hours post-dose

0 4 8 12 16 20 24

100 mg
300 mg
600 mg
1200 mg
Steady State PK and PD with Q12hr Dosing of BCX9930

BCX9930 PK & PD Profile at Steady State

BCX9930 PD Profile at 24 hr and Day 7 PK Troughs

AP Hemolysis

AP Wieslab

Day 1 Trough
Day 7 Trough
Day 1 Trough
Day 7 Trough
BCX9930 Phase 1 Trial: Summary

PK/PD
- Linear, dose-proportional exposure
- Dose-related suppression of alternative pathway of complement functional activity
- > 95% inhibition of alternative pathway in AP Wieslab assay at 100 mg Q12hr through 7 days of dosing

Safety & Tolerability
- 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 that included hepatic and renal
- Benign rash in majority of MAD subjects that was self-limited and resolved within a median of 5 days of onset
**BCX9930: Program Update**

- 12 healthy subjects (10 active, 2 placebo) vaccinated against *Neisseria meningitidis*, and then dosed with BCX9930 50 mg Q12h for a planned 14 days, in order to answer key scientific questions
- In the event of rash, subjects were to be discontinued from dosing if the rash was more than limited in extent
- Subjects with limited rash could continue on study drug per protocol
- Subjects who developed a rash could consent to skin biopsy

<table>
<thead>
<tr>
<th>Scientific question</th>
<th>Result</th>
<th>Conclusion</th>
</tr>
</thead>
<tbody>
<tr>
<td>Did antibiotic contribute to incidence of rash?</td>
<td>Benign rash observed in 7 subjects</td>
<td>NO— Antibiotic not likely a contributing factor</td>
</tr>
<tr>
<td>Was the clinical pattern of rash different in absence of antibiotic?</td>
<td>Same pattern was observed — clinically benign</td>
<td>NO— Antibiotic not likely a contributing factor</td>
</tr>
<tr>
<td>Did the rash change/worsen in subjects who continued dosing with study drug?</td>
<td>Same pattern clinically, median of 5 days duration</td>
<td>NO— In 2 healthy volunteers who continued dosing, rash resolved on-drug</td>
</tr>
<tr>
<td>Did biopsy results contribute to the understanding of the rash?</td>
<td>Majority of subjects with rash consented to biopsy - superficial perivascular dermatitis was found with no evidence of vasculitis</td>
<td>YES— Confirmed as benign</td>
</tr>
</tbody>
</table>

**Proof of Concept Data in PNH Patients Expected 1H 2020**
BCX9250
Oral ALK-2 Inhibitor for Fibrodysplasia Ossificans Progressiva (FOP)
Fibrodysplasia Ossificans Progressiva (FOP)
Devastating disease; no treatments available

- Rare disease that affects approximately 1 in 2 million people worldwide

- Irregular formation of bone in muscles, tendons or soft tissue

- Currently no approved treatments for FOP

- Phase 1 trial underway in healthy volunteers to assess safety, data expected 2H 2020
# Cash Position & 2019 Guidance (in Millions)

- *Added $100 M to balance sheet in Q4 2019 -*

<table>
<thead>
<tr>
<th>Description</th>
<th>Amount</th>
</tr>
</thead>
<tbody>
<tr>
<td>Cash &amp; investments at December 31, 2018</td>
<td>$128</td>
</tr>
<tr>
<td>Proforma cash &amp; investments at September 30, 2019&lt;sup&gt;A&lt;/sup&gt;</td>
<td>$170</td>
</tr>
<tr>
<td>Senior Credit Facility</td>
<td>$50</td>
</tr>
</tbody>
</table>

## FY 2019 GUIDANCE

<table>
<thead>
<tr>
<th>Description</th>
<th>Range</th>
</tr>
</thead>
<tbody>
<tr>
<td>Operating cash utilization</td>
<td>$105 – 130</td>
</tr>
<tr>
<td>Operating expenses&lt;sup&gt;B&lt;/sup&gt;</td>
<td>$120 – 145</td>
</tr>
</tbody>
</table>

---

<sup>A</sup> – Proforma cash balance adjusts Sept. 30, 2019 cash balance for $100 M of net proceeds from fourth quarter 2019 equity raises and upfront payment from Torii for Japanese commercial rights to berotralstat.

<sup>B</sup> - Excludes equity-based compensation.
The Year Ahead: Approvals, Launches and Data

2019 Accomplishments
- Submitted NDA to FDA for berotralstat
- Initiated oral Factor D Phase 1 trial for complement-mediated diseases
- Initiated oral ALK2 inhibitor Phase 1 study for development in FOP
- Added ~$100M in capital during 4Q 2019

2020 Priorities
1. Obtain berotralstat approvals in U.S. + Japan and submit MAA to EMA
2. Prepare commercial infrastructure for successful launches in the US & EU (+ support Torii in Japan)
3. Achieve proof of concept for oral Factor-D inhibitor in PNH patients
4. Continue advancing rare disease portfolio via in-house R&D or out-licensing partnerships