Forward-looking statement

BioCryst’s presentation may contain forward-looking statements, including statements regarding future results, unaudited and forward-looking financial information and company performance or achievements. Although product candidates, including BCX7353, may demonstrate promising results in early preclinical studies and clinical trials, there can be no assurance that any candidate will prove to be safe and effective in subsequent studies or trials. In addition, there can be no assurance that the results of development will lead to an NDA submission or approval or that any product will be commercially successful. 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://investor.shareholder.com/biocryst/sec.cfm
BioCryst’s strategy is to develop oral drugs for rare diseases

Drug discovery through structure-based design

- Lead optimization underway for two additional rare disease targets

Significant supporting capital from antiviral programs

- RAPIVAB® (peramivir injection) and Galidesivir
- Externally funded
- Stockpiling and voucher potential

Oral Drugs For Rare Diseases

Help patients lead normal lives

BCX7353 on track to report results for hereditary angioedema (HAE) in 1Q 2017
What makes BioCryst different?

Experienced drug discovery group (average tenure >15 years) focused on structure-based drug design

- Molecules built based on shape and charge of active site using iterative process
- Potent and specific enzyme blockers of challenging targets (e.g., Serine proteases, kinases, etc.)
- Ability to generate different classes of candidates quickly and efficiently
- Emphasize validated scientific targets to decrease risk
Evidence of in vitro potency and specificity

<table>
<thead>
<tr>
<th>RAPIVAB® (peramivir injection)</th>
<th>Galidesivir</th>
<th>BCX7353</th>
</tr>
</thead>
<tbody>
<tr>
<td>Neuraminidase enzyme assay</td>
<td>IC₅₀</td>
<td>EC₅₀ or IC₅₀</td>
</tr>
<tr>
<td>Influenza A/H1N1 strains NA²</td>
<td>0.01-1.8 nM</td>
<td>11.8 µM</td>
</tr>
<tr>
<td>Influenza B strains NA²</td>
<td>0.04 – 54 nM</td>
<td>4.4-6.7 µM</td>
</tr>
<tr>
<td>Mammalian NA³</td>
<td>&gt; 300 µM</td>
<td>&gt; 100 µM</td>
</tr>
<tr>
<td>Bacterial NA³</td>
<td>&gt; 300 µM</td>
<td>&gt; 100 µM</td>
</tr>
<tr>
<td>Parainfluenza viral NA³</td>
<td>&gt; 300 µM</td>
<td>&gt; 100 µM</td>
</tr>
</tbody>
</table>

2. RAPIVAB Package Insert
BioCryst’s pipeline

<table>
<thead>
<tr>
<th>STRATEGY: Develop oral therapies for life-threatening, rare diseases</th>
</tr>
</thead>
<tbody>
<tr>
<td>BCX7353 (HAE)</td>
</tr>
<tr>
<td>Next generation kallikrein inhibitors</td>
</tr>
<tr>
<td>Rare disease 1</td>
</tr>
<tr>
<td>Rare disease 2</td>
</tr>
</tbody>
</table>

**SUPPORTING ASSETS: Externally funded, potential for significant capital infusions**

<table>
<thead>
<tr>
<th>RAPIVAB® (peramivir injection)*</th>
<th>Lead optimization</th>
<th>Pre-clinical</th>
<th>Ph 1</th>
<th>Ph 2</th>
<th>Ph 3</th>
<th>Filed</th>
<th>Approved</th>
</tr>
</thead>
<tbody>
<tr>
<td>Galidesivir (broad spectrum antiviral) I.M.</td>
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<tr>
<td>Galidesivir (broad spectrum antiviral) I.V.</td>
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</tbody>
</table>

*licensed to Seqirus, Shionogi, & Green Cross in various geographies—additional filings anticipated
First target in strategy: Hereditary angioedema (HAE) is a high-need, high-value disease

Unpredictable, debilitating, potentially life-threatening swelling attacks

- Rare (estimated global prevalence of 1:50K)
- Growing US market ($1.2B, 30% growth in 2015)
- Significant global upside as paradigm shifts to attack prevention

Contact activation pathway is unbalanced based on genetic deficiency of C1 inhibitor (C1-INH)

- Plasma-derived C1-INH (chronic and acute, infusion)
- Androgen steroids (chronic, oral)
- Bradykinin inhibitor (acute, injection)
- Kallikrein inhibitor (acute, injection)

High-value, growing market on track to exceed $2.0B globally

Current standard of care therapies are injected/infused

Images obtained from www.haeimages.com
Market estimates based on analyst reports, earnings reports, and market data
Patients with HAE overwhelmingly prefer convenient oral therapy

Preferred route of administration among US HAE patients currently taking prophylactic therapy (N=83)

- 72% One pill, once daily
- 23% Subcutaneous injection (under the skin) every 4 weeks
- 5% Intravenous (injection in vein) 1 or 2 times per week

Question: Which medication would you prefer assuming each is equally safe and effective and costs the same amount per year?

C1 inhibitor levels in healthy people confirm the target range for restoring the normal phenotype of kallikrein inhibition in patients with HAE

**Hypothesis:** Increasing C1INH levels to > lower limit of normal (LLN) should eliminate angioedema attacks in HAE patients

**Questions:**

1. How much drug is needed to maintain kallikrein inhibition equivalent to > LLN for C1INH?
2. With higher drug doses, do we maintain higher blood levels?
3. Does maintaining higher blood levels give better response rates in HAE patients?
4. Can daily oral dosing with BCX7353 maintain the drug levels needed?
5. What proportion of patients could be expected to achieve these drug levels with daily oral dosing of BCX7353?
Higher trough levels of total C1INH (endogenous + dosed) during twice weekly administration for prophylaxis of HAE are associated with better efficacy.

**Dose-exposure analysis of SC C1INH**

**Estimated exposure-response for C1INH**

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**Sources:** Zuraw, B. L. *et al.* *Allergy* 70, 1319-1328, FDA Clinical Review (Cinryze), Cinryze label, CSL presentation at ACAAI 2016
Daily oral dosing with BCX7353 in healthy subjects achieves trough levels that meet or exceed the target range for efficacy.

In Vitro Activity: BCX7353 blocks HK cleavage in HAE plasma

100nM of BCX7353 (6.3 X EC$_{50}$ ratio) inhibits essentially all EAA-stimulated HK cleavage in HAE plasma

*multiples of EC$_{50}$ in fluorometric plasma kallikrein inhibition assay (15.9 nM)

Methods
94 μL of fresh HAE patient plasma + 0.15 μL of ellagic acid (EAA) + 1 μL of 7353, incubated for 5 minutes to activate contact pathway. Reaction was stopped and applied on the gel.
BCX7353 was generally safe and well tolerated over the range of doses and durations tested in Phase I

**Single doses of 10 mg through 1000 mg**

- No SAEs
- No clinically significant laboratory abnormalities
- 31 of 34 AEs were mild (grade 1)
- Three grade 2 events:
  - 1 subject in 100 mg cohort with moderate (grade 2) nausea and vomiting (2 AEs)
  - 1 subject in 100 mg cohort with moderate (grade 2) hay fever

**Once daily doses of 125 mg, 250 mg and 500 mg for 7 days; 350 mg for 14 days**

- No SAEs
- No clinically significant laboratory abnormalities
- 48 of 54 AEs were mild (grade 1)
- Five grade 2 events and 1 grade 3 event:
  - 350 mg QD x 14d cohort: 1 subject grade 2 upper abdominal pain (discontinued from study)
  - 500 mg QD x 7d cohort: 1 subject grade 2 syncope, 1 subject grade 2 headache, 1 subject grade 2 diarrhea and upper abdominal pain (2 AEs, discontinued from study), 1 subject grade 3 hypersensitivity reaction
Phase 2 placebo-controlled BCX7353 trial enrolling HAE patients

**Design**
- **Part 1**: proof of concept
  - 350 mg QD BCX7353 vs placebo
  - Interim analysis at n = 24
  - Option to add up to 12 subjects for total n = 36
  - Powered at 90% ($\alpha=0.05$) to detect a reduction in number of HAE attacks of $\geq 70\%$ on BCX7353
- **Part 2**: dose ranging
  - 250 mg QD and 125 mg QD BCX7353 and placebo
  - n = 14
  - 6:6:2 randomization

**Endpoints**
- Number of HAE attacks by treatment group will be analyzed as weekly attack rate, number of attacks, proportion of subjects with no attacks, number of attack-free days
- Additional endpoints include full safety assessments, QOL, PK/PD
BCX7353 Update

APeX-1 Update*

- 44 patients screened with 5 screen failures
- 34 patients randomized
- Recruiting continues, trial remains blinded, part 1 on track to report results in 1Q17

Estimated timing of key activities to support NDA/MAA filing

<table>
<thead>
<tr>
<th>H1 2017</th>
<th>H2 2017</th>
<th>2018</th>
<th>2019</th>
</tr>
</thead>
<tbody>
<tr>
<td></td>
<td></td>
<td>Phase 3 Efficacy - Safety Studies</td>
<td></td>
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<tr>
<td></td>
<td></td>
<td>Long Term Clinical Safety - Open Access Study</td>
<td></td>
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<tr>
<td></td>
<td></td>
<td>Nonclinical Carcinogenicity Studies</td>
<td></td>
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<tr>
<td></td>
<td></td>
<td>Drug Substance and Drug Product development and manufacturing</td>
<td></td>
</tr>
</tbody>
</table>

*As of January 6, 2017
Antiviral programs are externally funded, generating $400M to date with the potential for significant future capital infusions.

<table>
<thead>
<tr>
<th>Antiviral Program</th>
<th>Indication</th>
<th>Development funding</th>
<th>Additional capital infusions</th>
</tr>
</thead>
<tbody>
<tr>
<td>Galidesivir (BCX4430)</td>
<td>First and only one-dose IV treatment for influenza</td>
<td>Over $200M US Government funding to support development and approval</td>
<td>• Over $90M in milestones and royalty monetization</td>
</tr>
<tr>
<td></td>
<td>• Ebola is lead indication</td>
<td></td>
<td>• Over $25M in Government stockpiling (Japan/US)</td>
</tr>
<tr>
<td></td>
<td>• Broad-spectrum activity in Zika, Marburg and several other virus families</td>
<td>Approximately $80M US Government contract development funding</td>
<td>• Potential for Government stockpiling prior to FDA approval</td>
</tr>
<tr>
<td></td>
<td></td>
<td></td>
<td>• Eligible for FDA priority review voucher upon approval</td>
</tr>
</tbody>
</table>

Broad-spectrum activity increases attractiveness of Galidesivir for Government stockpiling.
Galidesivir path to stockpiling and NDA

- Outstanding animal survival data in Ebola, Marburg, Zika viruses
- Generally safe and well tolerated in phase 1 human study
- Clinical scale manufacturing

What’s Left

- NHP (pivotal) trials with IV administration
- Large (~200) healthy volunteer safety study
- Commercial scale manufacturing
### Precedent highly pathogenic countermeasures

<table>
<thead>
<tr>
<th>Product</th>
<th>Pathogen</th>
<th>Company</th>
<th>Doses</th>
<th>Cost</th>
</tr>
</thead>
<tbody>
<tr>
<td>BioThrax vaccine</td>
<td>Anthrax</td>
<td>Emergent BioSolutions</td>
<td>29M</td>
<td>$691M</td>
</tr>
<tr>
<td>Raxibacumab antitoxin (CY ’13)</td>
<td>Anthrax</td>
<td>GSK</td>
<td>60K</td>
<td>$193M</td>
</tr>
<tr>
<td>AbThrax antibody</td>
<td>Anthrax</td>
<td>HGS (now GSK)</td>
<td>65K</td>
<td>$326M</td>
</tr>
<tr>
<td>Botulimun antitoxin</td>
<td>Botulism</td>
<td>Cangene</td>
<td>200K</td>
<td>$427M</td>
</tr>
<tr>
<td>MVA vaccine</td>
<td>Smallpox</td>
<td>Bavarian Nordic</td>
<td>20M</td>
<td>$505M</td>
</tr>
<tr>
<td>ACAM2000 vaccine (CY ‘08)</td>
<td>Smallpox</td>
<td>Acambis</td>
<td>&gt;72M</td>
<td>$425M-$660M</td>
</tr>
<tr>
<td>ST-246 antiviral</td>
<td>Smallpox</td>
<td>Siga</td>
<td>1.7M</td>
<td>$433M</td>
</tr>
</tbody>
</table>

### Precedent voucher purchases

<table>
<thead>
<tr>
<th>Disease</th>
<th>Drug</th>
<th>Seller (Buyer)</th>
<th>Price</th>
</tr>
</thead>
<tbody>
<tr>
<td>Morquio A syndrome</td>
<td>Vimizim (elosulfase alfa)</td>
<td>BioMarin (Sanofi)</td>
<td>$67.5M</td>
</tr>
<tr>
<td>Leishmaniasis</td>
<td>Impavido (miltefosine)</td>
<td>Knight (Gilead)</td>
<td>$125M</td>
</tr>
<tr>
<td>High-risk neuroblastoma</td>
<td>Unituxin (dinutuximab)</td>
<td>United Therapeutics (Abbvie)</td>
<td>$350M</td>
</tr>
<tr>
<td>Rare bile acid synthesis</td>
<td>Cholbam</td>
<td>Retrophin (Sanofi)</td>
<td>$245M</td>
</tr>
</tbody>
</table>

Stockpiling data from FY2014 Budget for Public Health and Social Services Emergency Fund, pg 116
Voucher data sourced from public reports
# Cash position & 2016 guidance (in millions)

<p>| | |</p>
<table>
<thead>
<tr>
<th></th>
<th></th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>Cash &amp; investments at December 31, 2015</strong></td>
<td>$101</td>
</tr>
<tr>
<td><strong>Cash &amp; investments at December 31, 2016 (unaudited)</strong></td>
<td>$65</td>
</tr>
<tr>
<td><strong>Senior Credit Facility</strong></td>
<td>$23</td>
</tr>
<tr>
<td><strong>Cash runway</strong></td>
<td>Early 2018</td>
</tr>
</tbody>
</table>

**Guidance for 2016***:

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<table>
<thead>
<tr>
<th></th>
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</tr>
</thead>
<tbody>
<tr>
<td><strong>Operating cash utilization</strong></td>
<td>$55 – 75</td>
</tr>
<tr>
<td><strong>Operating expenses</strong>*#</td>
<td>$68 – 80</td>
</tr>
</tbody>
</table>

* Guidance for 2017 will be provided with our 2016 audited financial results later in Q1 2017

# Excludes equity-based compensation, and represents a modification from the previous range of $78 - 98 million
Summary: Building a company with the potential to generate expanding and sustainable value

Drug discovery through structure-based design

Significant supporting capital from antiviral programs

Oral Drugs For Rare Diseases

BioCryst Strategy

New Targets

HAE