Forward-Looking Statements

This presentation includes “forward-looking statements” within the meaning of the Private Securities Litigation Reform Act of 1995. Forward-looking statements in this presentation include, but are not limited to, statements that relate to the advancement and development of BXCL501 and BXCL701, the commencement of clinical trials, the availability and results of data from clinical trials, BioXcel Therapeutic, Inc.’s (“BTI”) submission of its first New Drug Application with the FDA and other information that is not historical information. When used herein, words including “anticipate”, “being”, “will”, “plan”, “may”, “continue”, and similar expressions are intended to identify forward-looking statements. In addition, any statements or information that refer to expectations, beliefs, plans, projections, objectives, performance or other characterizations of future events or circumstances, including any underlying assumptions, are forward-looking. All forward-looking statements are based upon BTI’s current expectations and various assumptions. BTI believes there is a reasonable basis for its expectations and beliefs, but they are inherently uncertain.

BTI may not realize its expectations, and its beliefs may not prove correct. Actual results could differ materially from those described or implied by such forward-looking statements as a result of various important factors, including, without limitation, its limited operating history; its incurrence of significant losses; its need for substantial additional funding and ability to raise capital when needed; its limited experience in drug discovery and drug development; its dependence on the success and commercialization of BXCL501 and BXCL701 and other product candidates; its ability to receive regulatory approval for its product candidates; its ability to enroll patients in its clinical trials; its approach to the discovery and development of product candidates based on EvolverAI is novel and unproven; its exposure to patent infringement lawsuits; its ability to comply with the extensive regulations applicable to it; its ability to commercialize its product candidates; and the other important factors discussed under the caption “Risk Factors” in its Quarterly Report on Form 10-Q for the period ended March 31, 2019 as such factors may be updated from time to time in its other filings with the SEC, which are accessible on the SEC’s website at www.sec.gov.

These and other important factors could cause actual results to differ materially from those indicated by the forward-looking statements made in this presentation. Any such forward-looking statements represent management’s estimates as of the date of this presentation. While BTI may elect to update such forward-looking statements at some point in the future, except as required by law, it disclaims any obligation to do so, even if subsequent events cause our views to change. These forward-looking statements should not be relied upon as representing BTI's views as of any date subsequent to the date of this presentation.
BioXcel Therapeutics Investment Highlights

Developing high value therapeutics in neuroscience and immuno-oncology utilizing a novel artificial intelligence platform

**Neuro Symptoms Based Approach**

**BXCL501**  
Sublingual Thin Film for Acute Treatment of Agitation

**Innate Immunity Based Approach**

**BXCL701**  
Targeting Rare Cancers

Clinical Partnerships

**AI-Powered Drug Development**

- Improves R&D Economics
- Increases Development Efficiency
- Maximizes Probability of Success
BTI is Unleashing the Power of AI Across the Entire R&D Value Chain

Opportunity to generate multiple NDAs

4-5 Year Development Cycle

1. MECHANISTIC INSIGHTS
   - Selection of Best Candidates

2. CLINICAL DEVELOPMENT TEAM
   - Human Proof Of Concept

3. TRANSLATIONAL TEAM
   - Candidate Validation

4. REGULATORY TEAM
   - Registration Filings

AI Powered Drug Development

NDA

BXCL501, BXCL701
BioXcel Therapeutics Pipeline: Rapid Human PoC and Development Path

First-in-class neuroscience and immuno-oncology pipeline with multiple near-term milestones

### Program

<table>
<thead>
<tr>
<th>Pipeline Expansion</th>
<th>Product Candidate</th>
<th>Phase 1/2</th>
<th>Phase 2/3</th>
<th>Anticipated Milestones</th>
<th>Worldwide Rights</th>
</tr>
</thead>
<tbody>
<tr>
<td></td>
<td><strong>BXCL501</strong></td>
<td>Pharmacokinetic &amp; Safety Study</td>
<td>Schizophrenia/Bipolar, Geriatric Dementia</td>
<td>✅ Initiated tNEPC phase 1b/2 trial (4Q 2018)</td>
<td>• New indications &amp; geography expansion (2019)</td>
</tr>
<tr>
<td></td>
<td><strong>BXCL701</strong></td>
<td>Opioid Withdrawal, Delirium, Exploring Multiple Tumor Types</td>
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<tr>
<td></td>
<td><strong>BXCL501</strong></td>
<td>Neuroendocrine Prostate Cancer (tNEPC) (double combination)</td>
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<tr>
<td></td>
<td><strong>BXCL701</strong></td>
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</tbody>
</table>

### Immuno-Oncology

- **BXCL501** (Selective α₂a Adrenergic Receptor Agonist)
  - Treatment of Acute Agitation
  - Phase 1b/2 trial initiation (4Q 2018)
  - Initiate pancreatic trials (1H 2019)
  - Preliminary tNEPC readout (2H 2019)
  - Preliminary pancreatic readouts (2H 2019)

- **BXCL701** (DPP 8/9 & FAP Inhibitor)
  - Initiated prostate phase 1b/2 trial (4Q 2018)
  - Initiate pancreatic trials (1H 2019)
  - Preliminary prostate readout (2H 2019)

### Future Programs

- Additional Discovery Through an Exclusive AI Relationship with BioXcel Corporation (parent)
  - Bioavailability (BA) study for optimizing BXCL501 sublingual thin film dose for Phase 3 registration trials

*Bioavailability (BA) study for optimizing BXCL501 sublingual thin film dose for Phase 3 registration trials
Clinical Programs

**BXCL501: First in Class Sublingual Thin Film Dexmedetomidine (Dex) for Acute Treatment of Agitation**
Current **Treatments are Suboptimal:**

- **Dementia:** Antipsychotic drugs (black-box warning) for elderly
- **Psychiatric:** Invasive with severe side effects

**BXCL501:** An innovative approach:

- Novel mechanism of action (MoA) targets a causal agitation pathway
- Non-Invasive, easy to administer **sublingual film** with **rapid onset of action**

**Consensus Opinion**

- Non-invasive
- Calmness without sedation
- Easy to administer
- Rapid onset
- Non-traumatic / non-coercive
- Good safety profile
- Favorable tolerability
- Patient preference

*1st International Experts’ Meeting on Agitation: Conclusions Regarding the Current and Ideal Management Paradigm of Agitation, Frontiers in Psychiatry 2018*
Treatment Across the Agitation Continuum

Targeting Moderate Agitation Key To Improving Care

- **AGITATION SEVERITY**
  - Mild
  - Moderate
  - Severe

- **PRESENTING PATIENT**
  - Cooperative
  - Uncooperative

- **MEDICATION OPTIONS**
  - Pill
  - Injection + Restraint

- **MEDICAL NEED**

  **ESCALATING AGITATION**
  - Rapid symptom control desirable
  - Pills too slow/injections too invasive

Unmet Medical Need & Commercial Opportunity
Proprietary Sublingual Thin Film Technology

Automated process for scale up to Phase III and commercial readiness

Phase 3 and Commercial Readiness

- Transitioned to automated manufacturing
- GMP automated manufacture initiated
- Scale up and supply phase 3 in 2H 2019 and commercial readiness in 2020

Ideal Pharmaceutical Properties for a Non-invasive Sublingual Film Formulation

Film manufacturing completed for clinical studies:

- Multiple dose strengths: 10µg to 60µg
- Immediate release film with muco-adhesion properties
- Proprietary technology delivers low dose ranges
Sublingual Film (BXCL501) PK and Safety Study

First In Human Study For Sublingual Thin Film Of Dexmedetomidine

Characterize Exposure Levels and Define Therapeutic Doses

- Double blind, placebo-controlled, single ascending dose, PK study
  - Healthy adult volunteers ages 18-65 (N = 42, 20 female)
  - Single center study
  - 3 Doses: 10, 20, 40 μg

- Primary objective:
  - Determine PK, safety and tolerability of various film strengths
BXCL501 Rapidly Achieved Targeted Exposures

BXCL501 Exhibited Predictable PK

- Rapidly delivered targeted exposures
  - Consistent with therapeutic responses seen in the IV Dex schizophrenia study

- Predictable and dose proportional PK
  - Enables dose selection for future development

- Pharmacodynamic (PD) effects lasted 4-6 hours
  - Optimal treatment duration

* Estimated concentration level based on Company observations in prior IV Dex study.
BXCL501 was Well Tolerated

Consistent Safety Profile Between Sublingual Film And IV Dex

- No serious adverse events (AEs)
- All AEs were Grade 2 or below (mild to moderate) and transient
  - Most common AE was drowsiness, observed at rates similar to placebo
  - Cardiovascular changes were not clinically meaningful
- No clear sedative effect for treatment group vs. placebo
- Maximum tolerated dose was not reached
Positive Human Proof of Concept: Acute Reduction of Agitation in 4 Indications

Safety Profile And Exposure Levels Were Consistent Across Indications With IV Dex

**DELIRIUM**
- 132 patients (46 refractory to haloperidol)
- **46/46 responded to IV Dex** in reducing agitation

*Carrasco et.al., Critical Care Medicine: July 2016, Vol 44, Issue 7, pp. 1295-1309

**SCHIZOPHRENIA**
- 14 patient study (10 treatment + 4 placebo)
- PEC/RASS scores indicate de-agitation without excessive sedation

**DEMENTIA**
- 14 patient study (10 treatment + 4 placebo)
- RASS* score of -1

**OPIOID WITHDRAWAL**
- 15 subject study (10 treatment + 5 placebo)
- 50% reduction in COWS total score

*PEC = Positive and Negative Symptom Scale-Excitatory Component

*RASS = Richmond Agitation Sedation Scale

*COWS = Clinical Opiate Withdrawal Scale
Human Proof of Concept: IV Dex Reduced Agitation in Schizophrenia Patients

Translating Efficacious Exposures From IV Dex To Sublingual Film

Study Design

- Randomized, placebo-controlled dose-ranging study
- 14 patients [10 treatment + 4 placebo]
- Primary endpoint: RASS of -1
- Secondary endpoint: PEC score of 7 or below

Early PEC Reduction Before Drowsiness

BXCL501 PK Study Exposures Consistent with Reduction in PEC Scores
### BXCL501 Integrated Clinical Development Plan

**Acute Agitation Studies: Short Duration With Easily Measurable Clinical Endpoints**

#### Randomized, Double-blind, Placebo-controlled Multi-center Studies

<table>
<thead>
<tr>
<th>Study Phase</th>
<th>Description</th>
<th>Treatment</th>
<th>Endpoint</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>PHASE 2 (US)</strong></td>
<td>Agitated Schizophrenia patients (N = ~200)</td>
<td>BXCL501</td>
<td>PRIMARY ENDPOINT: 2 HOURS Change from baseline on PANSS-Excitatory Component (PEC) rating scale</td>
</tr>
<tr>
<td></td>
<td></td>
<td>Placebo</td>
<td></td>
</tr>
<tr>
<td><strong>PHASE 3 (Global)</strong></td>
<td>Agitated Schizophrenia or Bipolar patients (N = ~700)</td>
<td>BXCL501</td>
<td>PRIMARY ENDPOINT: 2 HOURS Change from baseline on PANSS-Excitatory Component (PEC) rating scale</td>
</tr>
<tr>
<td></td>
<td></td>
<td>Placebo</td>
<td></td>
</tr>
</tbody>
</table>
Key Targeted Milestones for Value Creation

On Track For First NDA Submission In 2H 2020

BXCL501 Anticipated Timeline

<table>
<thead>
<tr>
<th>(Schizophrenia / Bipolar Disorder)</th>
<th>Phase 2 Trial Initiation</th>
<th>Phase 2 Data Readout</th>
<th>Phase 3 Trial Initiation</th>
<th>Phase 3 Data Readout</th>
<th>First NDA Submission</th>
</tr>
</thead>
<tbody>
<tr>
<td>2Q’19</td>
<td>3Q’19</td>
<td>4Q’19</td>
<td>1H’20</td>
<td>2H’20</td>
<td></td>
</tr>
</tbody>
</table>

Development plans for Agitated Dementia, Opioid Withdrawal and Delirium will be presented through 2019
BXCL501 US Commercial Opportunity

Target Patient Population Estimated at 3 Million

- 3 Million Patients With Moderate Agitation
- Multiple Episodes Per Year

Sources:
- Internal Company Estimates
- https://www.sccm.org/Communications/Critical-Care-Statistics
- https://www.samhsa.gov/data/
Clinical Programs

**BXCL701**: First-in-Class Oral IO Therapy Targeting Pancreatic Cancer and tNEPC
BXCL701: Potential First-in-Class Oral IO Therapy Targeting Pancreatic Cancer and tNEPC

Rare tumors with large market opportunity and limited competition

Orally Administered Activator of Systemic Innate Immunity Pathway

Dual MoA Inhibits DPP 8/9 & FAP

Established clinical proof of mechanism & tolerable safety profile (>700 patient data)

BXCL701 Human Proof of Concept & Mechanism of Action

With complementary factors and effects

 BXCL701 Human Proof of Concept

- Single Agent Activity in Melanoma
- ~10% Response Rate (CR/PR)
- Comparable to Yervoy (anti-CTL4)

>700
Patient Data

Inflammasome ↓
Caspase-1 ↓
Pyroptosis & IL-18 release

DPP 8/9

IMMUNE ACTIVATION

BXCL701

IMMUNE EVASION

FAP

Breaks Fibrotic Barrier

Patient Data

FXCL701

Single Agent Activity in Melanoma
~10% Response Rate (CR/PR)
Comparable to Yervoy (anti-CTL4)
Pancreatic Cancer Clinical Development Plan: Mechanistic and Anti-PD1 Combo Trial

Biomarker driven development in advanced pancreatic cancer, potential breakthrough designation

Proof of Mechanism Trial

2 Weeks of BXCL701 Treatment Before Surgery (Pre and Post Tissue Available (N=10-15))

BXCL701
NKTR-214
Avelumab

Efficacy Trial in Metastatic Patients after First-line Treatment

Triple Combination Phase 2 Expansion (N=30)

Simon 2-stage: 15+15
Primary Endpoint: ORR Combination: > 15%
Secondary Endpoint: DoR, PFS, OS
Exploratory Endpoint: Effect on immune cells (MDSC, T-cells, neutrophils)

Demonstration of Immune Cell Infiltration/Activation to Validate MoA

Louis Weiner, M.D.
Director

Global* Pivotal Study
tNEPC Clinical Development Plan: BXCL701 Combination with Keytruda

Biomarker driven development, breakthrough and fast track designation potential

Safety Run-in
Safety/PD/Immune-phenotyping (N=6)

Phase 2 Expansion
(N=30)

✔ Patient Recruitment Ongoing

✔ CTA Accepted 2Q19

Simon 2-stage: 15+15

**Primary Endpoint:** ORR Combination: increase from ~3-5% (Keytruda single agent) to > 15%

**Secondary Endpoint:** DoR, PFS, OS

**Exploratory Endpoint:** Effect on immune cells (MDSC, T-cells, neutrophils)

Global* Pivotal Study
*Expect to commence global development planning during Phase 2
Focus on EU and Japan

Eric Small, M.D.
Chief, Division of Hematology/Oncology

Johann de Bono, M.D., Ph.D.
Head, Division of Clinical Studies
Key Targeted Milestones for Value Creation

Data readouts expected into 1H 2020

**BXCL701**

Anticipated Timeline

<table>
<thead>
<tr>
<th>Neuroendocrine Prostate Cancer (tNEPC)</th>
<th>Combination Trial Initiated (BXCL701+Keytruda)</th>
<th>tNEPC Data Readout</th>
<th>Pancreatic Data Readouts</th>
<th>Registration Trial</th>
<th>Beyond</th>
</tr>
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<td>Pancreatic Data Readouts</td>
<td>Registration Trial</td>
<td>Beyond</td>
</tr>
</tbody>
</table>

- **Neuroendocrine Prostate Cancer (tNEPC)**
  - Combination Trial Initiated (BXCL701+Keytruda)
  - tNEPC Data Readout

- **Pancreatic Cancer (PDA)**
  - Triple Combination Trial Initiation
  - Mechanism Trial Initiation
  - Pancreatic Data Readouts

- Registration Trial
- NDA

**Anticipated Timeline**

- 2H’18
- 1H’19
- 2H’19
- 1H’20
- 2H’20
- Beyond
**Optimally Positioned for Execution**

*Support from world-class investors*

---

**Funded to Reach Multiple Inflection Points**

<table>
<thead>
<tr>
<th>Total Cash and Cash Equivalents:</th>
<th>36.3 million as of March 31(^{st}), 2019</th>
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<table>
<thead>
<tr>
<th>Major Shareholders:</th>
</tr>
</thead>
<tbody>
<tr>
<td>Artemis (7.4%)*</td>
</tr>
<tr>
<td>Fidelity (5.5%)*</td>
</tr>
<tr>
<td>DNCA Finance (7.32%)</td>
</tr>
</tbody>
</table>

<table>
<thead>
<tr>
<th>Analyst Coverage:</th>
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<tbody>
<tr>
<td>Geoff Meacham (Barclays)</td>
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<tr>
<td>Carter Gould (UBS)</td>
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<tr>
<td>Do Kim (BMO Capital Markets)</td>
</tr>
<tr>
<td>Sumant Kulkarni (Canaccord Genuity)</td>
</tr>
<tr>
<td>Ram Selvaraju (H.C. Wainwright)</td>
</tr>
</tbody>
</table>

* As of February 2019
Dr. Vimal Mehta, CEO
BioXcel Therapeutics, New Haven, CT 06511
vmehta@bioxceltherapeutics.com