AI-Driven Transformative Medicines in Neuroscience and Immuno-Oncology

May 2023
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 regarding BioXcel Therapeutics' expected timing of, and data results from, trials and clinical studies, and other milestones involving its product candidates including BXCL501, BXCL502, BXCL701 and BXCL702; planned discussions with regulators; its commercial plan, targets, and strategy for IGALMI™, including formulary approval at hospitals; and strategic options for OnkosXcel; potential benefits of treatment with BXCL501 and BXCL701, potential market size and opportunity for products and product candidates, and its future financial and operational results. 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 BioXcel Therapeutics' current expectations and various assumptions. BioXcel Therapeutics believes there is a reasonable basis for its expectations and beliefs, but they are inherently uncertain.

BioXcel Therapeutics 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 IGALMI™, BXCL501, BXCL502 and BXCL701 and other product candidates; the Company has no experience in marketing and selling drug products; IGALMI™ or the Company's product candidates may not be accepted by physicians or the medical community in general; the failure of preliminary data from its clinical studies to predict final study results; failure of its early clinical studies or preclinical studies to predict future clinical studies; its ability to receive regulatory approval for its product candidates; its ability to enroll patients in its clinical trials; undesirable side effects caused by the Company's product candidates; its novel approach to the discovery and development of product candidates based on EvolverAI; its exposure to patent infringement lawsuits; its ability to comply with the extensive regulations applicable to it; impacts from the COVID-19 pandemic; risks associated with the increased scrutiny related to environmental, social and governance (ESG) matters, its ability to commercialize its product candidates; and the other important factors discussed under the caption "Risk Factors" in its Annual Report on Form 10-K for the year ended December 31, 2022, as such factors may be further updated from time to time in its other filings with the SEC, including, but not limited to, its Quarterly Report for the quarterly period ended March 31, 2023, which are accessible on the SEC's website at www.sec.gov and the Investors section of our website at www.bioxceltherapeutics.com.

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 BioXcel Therapeutics 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 BioXcel Therapeutics' views as of any date subsequent to the date of this presentation.
**Indication and Important Safety Information**

**INDICATION**
IGALMI™ (dexmedetomidine) sublingual film is a prescription medicine, administered under the supervision of a health care provider, that is placed under the tongue or behind the lower lip and is used for the acute treatment of agitation associated with schizophrenia and bipolar disorder I or II in adults. The safety and effectiveness of IGALMI has not been studied beyond 24 hours from the first dose. It is not known if IGALMI is safe and effective in children.

**IMPORTANT SAFETY INFORMATION**
IGALMI can cause serious side effects, including:

- **Decreased blood pressure, low blood pressure upon standing, and slower than normal heart rate,** which may be more likely in patients with low blood volume, diabetes, chronic high blood pressure, and older patients. IGALMI is taken under the supervision of a healthcare provider who will monitor vital signs (like blood pressure and heart rate) and alertness after IGALMI is administered to help prevent falling or fainting. Patients should be adequately hydrated and sit or lie down after taking IGALMI and instructed to tell their healthcare provider if they feel dizzy, lightheaded, or faint.

- **Heart rhythm changes (QT interval prolongation).** IGALMI should not be given to patients with an abnormal heart rhythm, a history of an irregular heartbeat, slow heart rate, low potassium, low magnesium, or taking other drugs that could affect heart rhythm. Taking IGALMI with a history of abnormal heart rhythm can increase the risk of torsades de pointes and sudden death. Patients should be instructed to tell their healthcare provider immediately if they feel faint or have heart palpitations.

- **Sleepiness/drowsiness.** Patients should not perform activities requiring mental alertness, such as driving or operating hazardous machinery, for at least 8 hours after taking IGALMI.

- **Withdrawal reactions, tolerance, and decreased response/efficacy.** IGALMI was not studied for longer than 24 hours after the first dose. Physical dependence, withdrawal symptoms (e.g., nausea, vomiting, agitation), and decreased response to IGALMI may occur if IGALMI is used longer than 24 hours.

The most common side effects of IGALMI in clinical studies were sleepiness or drowsiness, a prickling or tingling sensation or numbness of the mouth, dizziness, dry mouth, low blood pressure, and low blood pressure upon standing.

These are not all the possible side effects of IGALMI. Patients should speak with their healthcare provider for medical advice about side effects.

Patients should tell their healthcare provider about their medical history, including if they suffer from any known heart problems, low potassium, low magnesium, low blood pressure, low heart rate, diabetes, high blood pressure, history of fainting, or liver impairment. They should also tell their healthcare provider if they are pregnant or breastfeeding or take any medicines, including prescription and over-the-counter medicines, vitamins, and herbal supplements. Patients should especially tell their healthcare provider if they take any drugs that lower blood pressure, change heart rate, or take anesthetics, sedatives, hypnotics, and opioids.

Everyone is encouraged to report negative side effects of prescription drugs to the FDA. Visit [www.fda.gov/medwatch](http://www.fda.gov/medwatch) or call 1-800-FDA-1088. You can also contact BioXcel Therapeutics, Inc. at 1-833-201-1088 or medinfo@bioxceltherapeutics.com.

Please see full Prescribing Information.
Contents

• Corporate Overview
• AI Innovation Engine
• Neuroscience Portfolio
  – IGALMI™ Commercialization
  – Neuroscience Portfolio Development Strategy
  – BXCL502
• Immuno-Oncology/OnkosXcel Therapeutics
• Upcoming Expected Milestones
Appendix
Corporate Overview
BioXcel Therapeutics: Targeting High Unmet Needs in Neuroscience and Immuno-Oncology

- Optimize R&D, accelerate development, increase probability of success

Neuroscience (BXCL501): First-in-human trials to FDA approval and launch in under 4 years

- IGALMI™ (dexmedetomidine) sublingual film, for the acute treatment of agitation in schizophrenia or bipolar I and II disorder in adults
- Multiple indications for BXCL501, $15B total potential market opportunity¹

Lead Oncology Drug Candidate: BXCL701

- Unique oral innate immune activator, designed to turn cold tumors hot via DPP8/9 inhibition
- Combination approach, BXCL701 plus KEYTRUDA® (penbrolizumab)
- Extend the value of immuno-oncology in large underserved patient populations
- Focusing on cold tumor types
- Positive Phase 2a data in SCNC presented at ASCO GU 2023

¹ Data on file. BioXcel Therapeutics, Inc. New Haven, CT December 2020 139M episodes @ $105/episode
Disruption is in Our DNA
Developing Transformative Medicines in Two Underserved Therapeutic Areas

Delivering innovation
Disrupting drug development paradigm
First drug approved and POC achieved with second drug candidate
$260m strategic financing in April 2022

First public AI company focused on neuroscience and immuno-oncology (2018)
Initial New Drug (IND) application to commercial launch of IGALMI™ in under 4 years
AI-based drug development and commercialization capability
Advanced commercial launch activities and clinical pipeline development

Neuroscience franchise
Poised to potentially capture 139-million-episode¹ U.S. agitation market
Poised to potentially impact cold solid tumor market

¹ Data on file. BioXcel Therapeutics, Inc., New Haven, CT; for Bipolar disorders, schizophrenia & Alzheimer’s-related agitation
<table>
<thead>
<tr>
<th>Compound</th>
<th>Indication/Proposed Indication</th>
<th>Preclinical</th>
<th>Phase 1</th>
<th>Phase 2</th>
<th>Phase 3</th>
<th>Registration</th>
<th>Marketed</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>Neuroscience</strong></td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Igalmi (demedetomidine)</td>
<td>Acute treatment of agitation associated with schizophrenia or bipolar I or II disorder in adults</td>
<td>Approved April 5, 2022</td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>BXCL501</td>
<td>At-home acute treatment of agitation associated with schizophrenia or bipolar I or II disorder in adults</td>
<td>SERENITY III</td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td></td>
<td>Acute treatment of agitation associated with Alzheimer’s disease*</td>
<td>TRANQUILITY II &amp; III</td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td></td>
<td>Adjunctive treatment in Major Depressive Disorder</td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>BXCL502</td>
<td>Chronic agitation in Alzheimer's disease</td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Wearable Device (+BXCL501)**</td>
<td>Pre &amp; post-agitation in dementia</td>
<td>Phase 0 device testing</td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td><strong>Immuno-Oncology</strong></td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>BXCL701</td>
<td>Small cell neuroendocrine prostate cancer</td>
<td>Combination with KEYTRUDA®</td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
</tbody>
</table>

Pipeline as of May 8, 2023

The safety and efficacy of investigational agents and/or investigational uses of approved products have not been established

*Includes intermittent chronic agitation

**Regulatory path to be determined; device + drug combination to be evaluated after further evaluation of predictive algorithm
Senior Management Team

Vimal Mehta, Ph.D.
Chief Executive Officer & Founder

Richard I. Steinhart
Senior Vice President &
Chief Financial Officer

Frank D. Yocca, Ph.D.
Chief Scientific Officer

Robert Risinger, M.D.
Chief Medical Officer, Neuroscience

Vincent J. O’Neill, M.D.
Senior Vice President &
Chief R&D Officer
OnkosXcel Therapeutics

Chetan D. Lathia, Ph.D.
Senior Vice President &
Head of Translational Medicine
Clinical Pharmacology and Regulatory Affairs

Matt Wiley
Senior Vice President &
Chief Commercial Officer

Javier Rodriguez
Senior Vice President, Chief Legal
Officer & Corporate Secretary
AI Innovation Engine
Uniquely Integrated Drug Discovery & Development Capability
Utilizing Proprietary AI Platform

1. **MULTIPLE CANDIDATES**
   - Selection of Most Viable Candidates

2. **TRANSLATIONAL TEAM**
   - Candidate Validation

3. **CLINICAL DEVELOPMENT TEAM**
   - Human Proof of Concept

4. **REGULATORY TEAM**
   - Registration Submissions

**BIG DATA**
- AI Platform

**REGISTRATION TRIALS**
- Human Proof of Concept

**NDA SUBMISSION**
- Registration Submissions
Unique AI Approach: New Product Concepts with Focus on the Stress Axis

STRESS

Stress alters function and activity of CNS pathways

Changes in CNS pathway function and activity of CNS pathways

MACHINE LEARNING CONNECTS:
- Behaviors (symptoms)
- Stress pathways
- Drug targets
- Drugs
Universe of Stress-related Symptoms, Targets & Drugs
AI-Driven Insights Through Dynamic Connectivity Map

- 76 Targets

- 2 Targets | 32 Drugs
  - Hallucinations
    - Akathisia
      - 1 Target | 9 Drugs
    - Restlessness
      - 22 Targets | 5 Drugs
    - Impulsiveness
      - 6 Targets | 43 Drugs
    - Irritability
      - 59 Targets | 10 Drugs
  - Agitation
    - 44 Targets | 5 Drugs
  - Psychosis
    - 1 Target | 9 Drugs
  - Delusions
    - 1 Target | 31 Drugs
  - Dementia
    - 46 Targets | 41 Drugs
  - Emotional Distress
    - 1 Target | 1 Drug
  - Sleep Disorders
    - 3 Targets | 1 Drug
  - Substance Abuse
    - 3 Targets | 78 Drugs
  - Mood Changes
    - 3 Targets | 1 Drug
  - Panic Disorder
    - 4 Targets | 29 Drugs
  - Social Anxiety
    - 1 Target | 4 Drugs
  - Suicidal Behaviour
    - 1 Target | 15 Drugs
  - Tremor
    - 3 Targets | 40 Drugs

- 6 Targets | 43 Drugs
- 59 Targets | 10 Drugs
- 3 Targets | 1 Drug
- 4 Targets | 29 Drugs
- 1 Target | 4 Drugs
- 1 Target | 15 Drugs
- 3 Targets | 40 Drugs

AI - Driven Insights Through Dynamic Connectivity Map
AI-Enabled Development Journey for BXCL501

Focus on discovery of novel pathways for stress-related symptoms

Build sustainable, innovative pipeline of CNS drug candidates
IND to IGALMI™ Launch in Under 4 Years: Proven Business Model
First AI-Derived, FDA-Approved Drug With Novel Mechanism of Action

Q4 2018
- FDA Acceptance of IND for BXCL501
  - Acute Treatment of Agitation

Mid-2020
- 2 Positive Pivotal Phase 3 Data Readouts
  - Schizophrenia & Bipolar Disorders

Q1 2021
- FDA Breakthrough Therapy Designation for BXCL501
  - Dementia

Q2 2021
- FDA Acceptance of NDA Filing for BXCL501
  - Schizophrenia & Bipolar Disorders

April 5, 2022
- FDA Approval

July 2022
- Commercial Launch
Neuroscience Portfolio
Leading in Neuroscience Drug Development & Commercialization

5-Year Vision for Growth

INIovation

Proprietary AI to Commercialization Capability

Neuroscienece Portfolio

BXCL501 Potential Pipeline Within a Product Candidate Building Robust Portfolio
• Stress-Related Behaviors (BXCL502)
• Neuro-rare Diseases

Commercialization

Innovative Agitation Therapy Commercially Launched
Well-Positioned to Address Significant U.S. Agitation Market Opportunity

$15B Potential Market Opportunity*

16M BPD/SCZ Institutional Episodes¹⁻³

139M Agitation Episodes¹⁻³

100M Alzheimer’s* Dementia Episodes¹

23M BPD/SCZ At-Home* Episodes¹⁻³

139M episodes @ $105/episode

*Investigational use; safety and efficacy not established


IGALMI™ Commercialization
Agitation: Relatively Common and Difficult-to-Manage

Debilitating for Patients and Threatening for Healthcare Providers

Characterized by recurring episodes

Symptoms differ by patient, vary between episodes, and range from mild to severe

Multi-billion-dollar healthcare burden

Best-practice guidelines recommend agitation be treated by:

- Behavioral calming techniques
- Verbal de-escalation
- Medications voluntarily accepted by patients without coercion; pharmacologic goal of calming without unarousable sedation

Current treatment approaches:

- May involve physically restraining patients
- Over-sedating therapies such as antipsychotics and benzodiazepines
- Antipsychotic drugs have black box warnings for elderly

IGALMI™ (dexmedetomidine) Sublingual Film
Approved for Acute Treatment of Agitation Associated with Schizophrenia or Bipolar I or II Disorder in Adults

First and only FDA-approved orally dissolving sublingual film with broad label covering mild, moderate, and severe agitation

Limitations of Use: The safety and effectiveness of IGALMI have not been established beyond 24 hours from the first dose. Please see Important Safety Information and full Prescribing Information at www.igalmi.com
Commercial Momentum Accelerating
$300m+ in Market Opportunity Unlocked or Scheduled to Vote*

✓ $55m in market opportunity unlocked via 130 total formulary wins to date*; over $255m in opportunity scheduled to vote.

✓ Surrogate uptake curve shows IGALMI can penetrate agitation market volume per hospital rapidly, at over 4% in just 6 months.

✓ Sales team has now reached over 75% of total targets.

✓ Media impressions to drive awareness and demand increased over 70% in 1Q 2023, and 1,200+ HCPs have attended peer influence programs.

Cumulative Market Potential ($000’s)

Note: Based on average episodes per hospital/bed.

*Data as of April 30, 2023
130 Hospital Formularies Won to Date*, with ~30% of IDN beds Approved or Scheduled to Vote

Key Performance Indicators

*Data as of April 30, 2023

Note: 100 IDN beds equals approximately 1,300 agitation episodes
Addressable Market Opportunity Launch to Date*
Unlocked $55m of Addressable Market to Penetrate, with $255m+ in Opportunity Scheduled to Vote

*Data as of April 30, 2023
Observed IGALMI™ Uptake in Order Volume Among Leading Hospitals

Example Below Shows Aggregated IGALMI Market Penetration for Multiple Hospitals Over 6 Months

* Each hospital has an addressable volume of approximately 4,000 episodes/year. n=5
Amplifying IGALMI™ Awareness Through Multi-Channel Approach

Print Advertising and Branded Conference Booth
Neuroscience Portfolio Development Strategy
Our Land and Expand Strategy

Development Expansion
Alzheimer’s Disease

TRANQUILITY II Trial
Pivotal

Geographic Expansion
EU
Japan

Potential Strategic Partnerships

SERENITY III Trial Expansion
Pivotal

Medical Setting Expansion
At-home
Significant Market Opportunity: At-Home & Alzheimer’s Agitation

Alzheimer’s Agitation Could Potentially Increase BXCL501 U.S. Market Opportunity by Over Six-fold

16M BPD/SCZ Institutional Episodes¹-³

23M BPD/SCZ At-Home Episodes¹-³

SERENITY III Pivotal Trial

100M Alzheimer’s Dementia Episodes³

TRANQUILITY Pivotal Program

~$15B Potential Market Opportunity*


*139M episodes @ $105/episode
SERENITY III Pivotal Trial
At-home use of BXCL501 for Acute Treatment of Bipolar or Schizophrenia-related Agitation

PART 1: EFFICACY
Patients with Agitation

Screening → BXCL501 (60 mcg) → Placebo N=50
1-Day In-Clinic Treatment
Primary endpoint: change from baseline in PEC score (PANSS-Excitatory Component)

PART 2: SAFETY
Patients with Agitation

Screening → BXCL501 (60 mcg) → Placebo N=50
12-Week At-Home Treatment
Primary endpoint: TEAEs collected by informant and clinical investigator in at-home environment
Significant Market-Expansion Opportunity: Alzheimer’s Disease

~100M agitation episodes per year in the U.S.¹

U.S. adults 65+ with AD to Double by 2040²

---

Clinically Meaningful, Rapid, and Durable Response Observed

TRANQUILITY I Trial

Change in PEC Score from Baseline

Change in PAS Score from Baseline

<table>
<thead>
<tr>
<th>Placebo</th>
<th>BXCL501 30 mcg</th>
<th>BXCL501 60 mcg</th>
</tr>
</thead>
<tbody>
<tr>
<td>Baseline</td>
<td>-2.5</td>
<td>-5.7</td>
</tr>
<tr>
<td>(LS Mean)</td>
<td></td>
<td></td>
</tr>
<tr>
<td>Response</td>
<td>0%</td>
<td>31%</td>
</tr>
</tbody>
</table>

P values at 0.5 hrs are 0.0295 for BXCL501 30 mcg and 0.0568 for BXCL501 60 mcg

Pittsburgh Agitation Scale (PAS) measures 4 behavior groups: aberrant vocalization, motor agitation, aggressiveness, and resisting to care rated 0– no agitation present to 4 – highest form of agitation.

ITT analysis, Least Square Means ± SEM

Proportion achieving ≥ 40% PEC reduction
TRANQUILITY II and TRANQUILITY III Pivotal Trials

Pivotal Program of BXCL501 for Acute Treatment of Agitation in Patients with Alzheimer’s Disease

**TRANQUILITY II:** Patients requiring **minimal assistance** in assisted living and residential care facilities

- **Screening**
- **R**

12-week dosing as needed
(Primary endpoint: PEC at 2 hours)

- BXCL501 (40 mcg) N=50
- BXCL501 (60 mcg) N=50
- Placebo N=50

**TRANQUILITY III:** Patients requiring **moderate assistance** in long-term care facilities

- **Screening**
- **R**

- BXCL501 (40 mcg) N=50
- BXCL501 (60 mcg) N=50
- Placebo N=50

**Rollover Safety Study**
Open-label, long-term, one-year safety study dosed as needed
Comprehensive Alzheimer’s Disease Program Strategy

Agitation Spectrum

- Pre-Agitation
- Acute Agitation
- Intermittent Agitation
- Chronic Agitation

Wearable + PRN

- PRN* BXCL501 PRN*
- BXCL502

Treatment Settings Spectrum

- Hospitals/EDs
- Assisted Living/Nursing Homes
- At Home

*As needed
Depression Represents a Considerable Societal Burden

- **300m+** Antidepressant prescriptions filled annually\(^1\)
- **15M+** Americans currently receive treatment for depression\(^2\)
- **12.7%** U.S. population over 12 years old took antidepressants in prior month\(^3\)
- **9%** 12-month prevalence of depression in U.S. population\(^4\)
- **25%** Remain ill 10 years after starting treatment

Sources:
1. IQVIA, 2021. This information is an estimate derived from the use of information under license from the following IQVIA information service: NPA for the period 2021. IQVIA expressly reserves all rights, including rights of copying, distribution and republication.
Major Depressive Disorder: Multiple Ascending Dose (MAD) Study With Concomitant Treatment With Antidepressant

Designed to Inform BXCL501 Dose Selection in Future Proof-of-Concept Study

<table>
<thead>
<tr>
<th>Cohorts 1 - 4*</th>
<th>HV Screening</th>
<th>7-Day dosing (qAM)</th>
</tr>
</thead>
<tbody>
<tr>
<td>(BXCL501: 30; 60; 80; 120 mcg)</td>
<td></td>
<td>BXCL501 N=12</td>
</tr>
<tr>
<td></td>
<td></td>
<td>Placebo N=6</td>
</tr>
</tbody>
</table>

*Cohorts to be completed sequentially

- Cohorts 1-4 completed (No dose-limiting AEs)

<table>
<thead>
<tr>
<th>Cohorts 5 - 6*</th>
<th>HV Screening</th>
<th>7-Day dosing (qAM+qPM)</th>
</tr>
</thead>
<tbody>
<tr>
<td>(BXCL501: 30+60; 40+80 mcg)</td>
<td></td>
<td>BXCL501 N=12</td>
</tr>
<tr>
<td></td>
<td></td>
<td>Placebo N=6</td>
</tr>
</tbody>
</table>

- Cohorts 5-6 completed (No dose-limiting AEs)

<table>
<thead>
<tr>
<th>Cohort 7</th>
<th>HV Screening</th>
<th>7-Day dosing</th>
</tr>
</thead>
<tbody>
<tr>
<td></td>
<td></td>
<td>Duloxetine BID + BXCL501 (MTD) qHS N=8</td>
</tr>
<tr>
<td></td>
<td></td>
<td>Placebo N=4</td>
</tr>
</tbody>
</table>

- Completed: BXCL501 MTD is adequately tolerated when given with an effective dose of an SNRI (duloxetine)
BXCL502
Stress alters the function and activity of CNS pathways

Changes in CNS pathway function and activity of CNS pathways

BXCL502
Targeted to specific brain regions that are activated by stress and result in troubling psychiatric symptoms
**BXCL502: a Differentiated, De-risked Candidate for Chronic Treatment of Stress-Related Disorders**

<table>
<thead>
<tr>
<th>Pathway</th>
<th>✓ High expression in brain on pathways associated with stress response</th>
</tr>
</thead>
<tbody>
<tr>
<td>Confidence and Rationale</td>
<td>✓ Robust confidence in rationale, based on preclinical studies that showed comparable activity to benzodiazepines and antidepressants</td>
</tr>
<tr>
<td>Efficacy Results</td>
<td>✓ Showed improvement in clinically validated endpoint for neuropsychiatric symptoms related to agitation</td>
</tr>
<tr>
<td>Human Safety</td>
<td>✓ Generally well-tolerated in hundreds of patients after 52 weeks of dosing</td>
</tr>
</tbody>
</table>
| Patent Strategy | ✓ Novel formulation strategy in development  
✓ Opportunities to expand IP position in combination with complementary mechanisms |

- **PROPOSED INDICATION:** Chronic treatment of agitation as a monotherapy in patients with dementia
- **Potential for treating anxiety disorders**
Immuno-Oncology

OnkosXcel Therapeutics™

A subsidiary of BioXcel Therapeutics, Inc.
Established OnkosXcel: Now is the Time

Value Creation
High-potential, dedicated oncology subsidiary with an efficient development path

Clear Focus
Hard-to-treat tumors with focus on innate immunity (BXCL701 lead asset with ~800-subject safety database) and utilizing artificial intelligence platform

Proven Expertise
Led by a world-class management team

Established Infrastructure
Well-positioned to deliver on key milestones and fulfill our mission
## BXCL701: Strong Value Proposition in Hard-to-Treat Tumors

Pipeline With a Product Potential

### Novel Proposed Mechanism of Action

**Data Published in JITC**

One of the most clinically advanced oral innate immune activators, designed to activate inflammasome via DPP8/9 inhibition

### Completed Phase 2a Data for SCNC

**Presented at ASCO GU 2023**

- Composite response rate: 25%
- Median duration of response: 6+ months*
- Generally well tolerated in combination with KEYTRUDA® (pembrolizumab)

### Clinical Proof of Concept

**Cold Tumors**

- Demonstrated positive efficacy results in two cancer types: mCRPC small cell neuroendocrine prostate cancer (SCNC) and adenocarcinoma
- ~800-subject clinical safety database

### Working to Develop Leadership Position

**in Innate Immunity DPP8/9 Biology**

Scarcity of assets in innate immunity

*As of data cutoff on December 19, 2022*
First AI-Derived Human POC for Oral Innate Immune Activator
Utilizing Extensive Data from 11 Prior Clinical Trials and ~700 Patients

Q4 2018
Received FDA allowance of IND for BXCL701 in SCNC

Q2 2020
Initiation of Phase 2 efficacy portion of Phase 1b/2 trial

Q4 2020
Initial data from Phase 1b/2 trial

Q1 2021
Update on efficacy data from Phase 1b/2 trial

Q1 2022
SCNC Phase 2a interim efficacy/safety data

Q3 2022
Durability of Phase 2a interim efficacy / safety data

Q1 2023
Complete SCNC Phase 2a efficacy / safety data

2H 2023
Phase 2b initiation

Acquired asset developed by Point Therapeutics (talabostat)

1 Timing and nature of the study subject to further discussions with FDA
## BXCL701: Pipeline Within a Product Plan and Next-Generation Candidate

<table>
<thead>
<tr>
<th>Compound</th>
<th>Proposed Indication</th>
<th>Preclinical</th>
<th>Phase 1</th>
<th>Phase 2</th>
<th>Phase 3</th>
<th>Expected Upcoming Milestone</th>
<th>Collaborator</th>
</tr>
</thead>
<tbody>
<tr>
<td>BXCL701</td>
<td>Small Cell Neuroendocrine Prostate Cancer (SCNC)</td>
<td></td>
<td></td>
<td></td>
<td></td>
<td>Initiate Phase 2b</td>
<td></td>
</tr>
<tr>
<td></td>
<td>Small Cell Lung Cancer (SCLC)</td>
<td></td>
<td></td>
<td></td>
<td></td>
<td>Initiate Phase 1b/2</td>
<td></td>
</tr>
<tr>
<td>BXCL701 ISTs*</td>
<td>Metastatic Pancreatic Ductal Adenocarcinoma</td>
<td></td>
<td></td>
<td></td>
<td></td>
<td>Phase 2 readout</td>
<td>supply agreement</td>
</tr>
<tr>
<td></td>
<td>Acute Myeloid Leukemia (AML)</td>
<td></td>
<td></td>
<td></td>
<td></td>
<td>Phase 1b readout</td>
<td>Dana-Farber Cancer Institute</td>
</tr>
<tr>
<td>Next-Generation DPP8/9 Inhibitor</td>
<td>Solid Tumors</td>
<td></td>
<td></td>
<td></td>
<td></td>
<td>Candidate nomination</td>
<td></td>
</tr>
</tbody>
</table>

* Investigator Sponsored Trials
Upcoming Expected Milestones
Three Expected Data Catalysts in Q2 2023

**NEUROSCIENCE: BXCL501**

- **Bipolar Disorders or Schizophrenia-Associated Agitation**
  - (At-home use)
  - Phase 3 SERENITY III Pivotal Trial Part 1
  - May 2023

- **Alzheimer’s-Associated Agitation**
  - Phase 3 TRANQUILITY II Pivotal Trial
  - June 2023

- **Major Depressive Disorder (MDD)**
  - Phase 1b MAD Trial in Healthy Volunteers
  - May 2023
Expected Clinical Trial Initiations

**NEUROSCIENCE: BXCL501**

Bipolar Disorders or Schizophrenia-Associated Agitation
(At-home use)
SERENITY III Part 2
Q2 2023

**IMMUNO-ONCOLOGY: BXCL701**

- Small Cell Neuroendocrine Prostate Cancer
  - Phase 2b Potential Pivotal Study\(^1\)
- Small Cell Lung Cancer
  - Phase 1b/2 Trial
2H 2023
2H 2023

---

\(^1\) Initial discussions with the FDA regarding the development pathway and registrational strategy for BXCL701 in SCNC expected in mid 2023
Appendix
Access and Demand Timeline Considerations

P&T Process can take 6-12 Months for each wave of sales hires; HCP product trial post-P&T approval can also take 6-12 Months.

P&T PROCESS

- Request for P&T Review
  - 0 – 3 Months
- P&T Review & IT Implementation
  - 3 – 12 Months

HCP DEMAND

- Product Trial First 6 Months (37%)
  - 12 – 18 Months
- Product Trial 6 – 12 Months (70%)
  - 18 – 24 Months

Wave 1: 26 Institutional Specialists
Wave 2: 44 Additional Institutional Specialists

Source: HCP ATU Landscape Research, January 2022; F7: Data presented is for Emergency Department HCPs. If PRODUCT X were available to you and on formulary, when would you most likely first try it from time of launch?
Thank you!