AI-Driven Transformative Medicines in Neuroscience and Immuno-oncology

41st Annual J.P. Morgan Healthcare Conference
January 11, 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 involving its product candidates; planned discussions with regulators; its commercial plan and strategy for IGALMI™ and strategic options for OnkosXcel; 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; 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 quarterly period ended September 30, 2022, 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 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
IGALMI is indicated for the acute treatment of agitation associated with schizophrenia or bipolar I or II disorder in adults. Limitations of Use: The safety and effectiveness of IGALMI have not been established beyond 24 hours from the first dose.

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 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.
Targeting High Unmet Needs in Neuroscience and Immuno-oncology

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

Neuroscience (BXCL501): FDA Approval

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

Immuno-oncology (BXCL701): Human Proof of Concept

- Unique innate immunity activator, turning cold tumors hot
- Established OnkosXcel Therapeutics to maximize value

High-Value Near-Term Catalysts

- 2 pivotal readouts for BXCL501 in 1H23
- Phase 2 readout for BXCL701 in 1Q23

Compelling Long-term Value

- Integrated AI-drug development & commercialization capability to build a leading neuroscience company
Disruption is in our DNA

- Delivering innovation
  - First public AI company focused on neuroscience and Immuno-oncology (2018)

- Disrupting drug development paradigm
  - IND to commercial launch of IGALMI™ in under 4 years

- First oral film treatment for BPD/SCZ agitation market
  - Built integrated commercial team and implementing dynamic launch model

- $260m strategic financing in April 2022
  - Advanced commercial launch activities and clinical pipeline development

- Poised to potentially capture 139-million-episode U.S. agitation market
  - Bipolar disorders, schizophrenia & Alzheimer’s-related agitation

Data on file. BioXcel Therapeutics, Inc. New Haven, CT
Uniquely Integrated Drug Discovery & Development Capability
Utilizing Proprietary AI Platform
Al-Driven Insights Into Universe of Stress-related Symptoms, Targets & Drugs

Dynamic Connectivity Map

- **HALLUCINATIONS**
  - 2 Targets | 32 Drugs
- **AKATHISIA**
  - 1 Target | 9 Drugs
- **AGITATION**
  - 44 Targets | 5 Drugs
- **PSYCHOSIS**
  - 1 Target | 9 Drugs
- **DELUSIONS**
  - 1 Target | 31 Drugs
- **DEMENTIA**
  - 46 Target | 41 Drugs
- **EMOTIONAL DISTRESS**
  - 1 Target | 1 Drug
- **SLEEP DISORDERS**
  - 3 Targets | 1 Drug
- **SUBSTANCE ABUSE**
  - 3 Targets | 78 Drugs
- **RESTLESSNESS**
  - 22 Targets | 5 Drugs
- **IMPULSIVENESS**
  - 6 Targets | 43 Drugs
- **IRRITABILITY**
  - 59 Targets | 10 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

76 Targets

22 Targets | 5 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
IND to IGALMI™ Launch in 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
Igalmi (dexmedetomidine) sublingual film: 120 mcg, 180 mcg

July 2022
**Innovation Shaping the Agitation Market**

Similar to Depression Market in the 1980s

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**Depression Market**

*The Philadelphia Inquirer*

“Drugs now treating depression generate more than a billion dollars in worldwide annual sales, financial analysts say. Before Prozac’s introduction in 1986, the U.S. anti-depressant market was relatively stagnant, generating about $280 million in annual sales, estimates.” Jerry Brimeyer, a drug analyst for Dean Witter Reynolds (1990)

*THE WALL STREET JOURNAL.*

“Psychiatrists say clinical depression still is badly underdiagnosed, suggesting the worldwide anti-depressant market could expand faster than its current annual rate of about 10%.” (1992)

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**IGALMI marketing and education efforts are highlighting the undervalued agitation market**

**Innovation driving market creation**

**Historically underdiagnosed and underserved markets**

**No commercial precedent or analogs**

*IGALMI* (DEXMEDETOMIDINE)

sublingual film - 120 mcg, 180 mcg
**Well-Positioned to Help Address Significant U.S. Market Opportunity**

*$15B Potential Market Opportunity$¹

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**Agitation Episodes¹⁻³**

- **139M**
- **100M** Alzheimer’s* Episodes¹

**23M** BPD/SCZ At-Home* Episodes¹⁻³

**16M** BPD/SCZ Institutional Episodes¹⁻³

**23M** BPD/SCZ At-Home* Episodes¹⁻³

**BXCL501**

**SERENITY III Pivotal Trial:**
Top-line Data Readout Expected in 1H 2023

**BXCL501**

**TRANQUILITY II**
Top-line Data Readout Expected in 1H 2023

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*139M episodes @ $105/episode  *Investigational use; safety and efficacy not established
# Potential Market-Changing Product & Current Pipeline

<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>
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<tbody>
<tr>
<td><strong>Neuroscience</strong></td>
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<tr>
<td>Igalmi (dermedetomidine)</td>
<td>Acute treatment of agitation associated with schizophrenia or bipolar I or II disorder in adults</td>
<td>Approved April 5, 2022</td>
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<td>BXCL501</td>
<td>At-home acute treatment of agitation associated with schizophrenia or bipolar I or II disorder in adults</td>
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<td>SERENITY III</td>
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<td></td>
<td>Acute treatment of agitation associated with Alzheimer’s disease*</td>
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<td>Adjunctive treatment in major depressive disorder</td>
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<td>BXCL502</td>
<td>Chronic agitation in Alzheimer’s disease</td>
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<td>Wearable Device (+BXCL501)**</td>
<td>Pre &amp; post-agitation in dementia</td>
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<td>Phase 0 device testing</td>
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<td><strong>Immuno-oncology</strong></td>
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<td>BXCL701</td>
<td>Small cell neuroendocrine metastatic castration-resistant prostate cancer</td>
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Pipeline as of January 11, 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
Four Data Catalysts on Track in 1H 2023

Anticipated near-term catalysts as of January 11, 2023

**NEUROSCIENCE: BXCL501**

- Alzheimer’s-Associated Agitation
  - Phase 3 TRANQUILITY II Pivotal Trial
- Bipolar Disorders or Schizophrenia-Associated Agitation (at-home use)
  - Phase 3 SERENITY III Pivotal Trial
- Major Depressive Disorder (MDD)
  - Phase 1 Dose Selection Trial in Healthy Volunteers

**IMMUNO-ONCOLOGY: BXCL701**

- Aggressive Variant of Metastatic Castration-Resistant Prostate Cancer
  - Phase 1b/2 Study
- Final efficacy data
  - 28-patient Phase 2a SCNC cohort
IGALMI™ Commercial Momentum
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

IGALMI profile represents significant game-changing market potential
IGALMI™: Poised for Success

2022

- 70 Reps
- Corporate Account Director Team Onboard
- Limited Marketing
- Initial Speaker Programs
- No Free Trial Program
- Building Data Lake

2023

- 26 Reps
- No field account team
- Enhanced Advertising to Raise Awareness
- Amplified Speaker Programs in H1
- Free Trial Program
- Leveraging Data Lake
Positive Commercial Momentum

Well-positioned to Maximize IGALMI™ Market Potential

• **Market reception** positive from key hospital stakeholders

• **Market dynamics** highly favorable to IGALMI value proposition

• **Market access** accelerating across multiple institutions

• **Full commercial infrastructure** to cover ~1700 hospitals in 70 geographies

**Strong momentum heading into 2023**
Perspectives From an Early Adopter Hospital

Koth Cassavaugh, Pharm.D.
Director of Pharmacy, Auburn Community Hospital
Auburn, NY

Speaker is acting on behalf of and is a paid consultant to BioXcel Therapeutics, Inc.
This material is intended for an investor audience only.
The information contained in the following material is intended to provide background and educational information only and does not constitute medical advice. Individual results will vary among patients and depend on many factors. A patient’s healthcare provider should consider the circumstances of each patient.
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Alzheimer’s Related Agitation

Breakthrough Therapy Designation
Our Land and Expand Strategy

- Development Expansion
  Alzheimer’s Disease

- TRANQUILITY II Trial Pivotal

- SERENITY III Trial Pivotal
  Medical Setting Expansion
  At-home

- Geographic Expansion
  EU
  Japan

- Strategic Partnerships
Alzheimer’s Program: Addressing Entire Agitation Spectrum

Pre-Agitation

Wearable + PRN

At Home

Treatment Settings Spectrum

Assisted Living/Nursing Homes

Market Entry Strategy Under Development

Acute Agitation

Intermittent Agitation

Chronic Agitation

PRN* BXCL501 PRN*

BXCL502
Significant Market Opportunity: At-home & Alzheimer’s Agitation

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

16M
BPD/SCZ
Institutional Episodes

23M
BPD/SCZ
At-Home Episodes

100M
Alzheimer’s Dementia Episodes

SERENITY III
Pivotal Trial

TRANQUILITY
Pivotal Program

~$15B
Potential Market Opportunity*


*139M episodes @ $105/episode
Established OnkosXcel: Now is the Time

<table>
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<tr>
<th><strong>Value Creation</strong></th>
<th>High-potential, dedicated oncology subsidiary with an efficient development path</th>
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<tr>
<td><strong>Clear Focus</strong></td>
<td>Hard-to-treat tumors with focus on innate immunity (BXCL701 lead asset with ~800-subject safety database) and utilizing artificial intelligence platform</td>
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<td><strong>Proven Expertise</strong></td>
<td>Led by a world-class management team</td>
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<tr>
<td><strong>Established Infrastructure</strong></td>
<td>Well-positioned to deliver on key milestones and fulfill our mission</td>
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## BXCL701: Strong Value Proposition in Hard-to-Treat Tumors

Distinct Leadership in Innate Immunity DPP8/9 Biology

<table>
<thead>
<tr>
<th>Mechanism of Action Published in JITC</th>
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<tr>
<td>One of most advanced oral innate immune activators activates inflammasome via DPP8/9 inhibition</td>
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<th>Clinical Proof of Concept Cold Tumors</th>
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<tr>
<td>Demonstrated efficacy in rare cancer: small cell neuroendocrine prostate cancer (SCNC)</td>
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<th>Distinct Position in Innate Immunity DPP8/9 Biology</th>
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<tr>
<td>Scarcity of assets in innate immunity</td>
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<th>Full Data Set for SCNC at ASCO GU</th>
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<tr>
<td>Response rate: 33%*</td>
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<td>Mean duration of response: 9 months</td>
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<td>Generally well tolerated in combination with KEYTRUDA®</td>
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*First 15 evaluable patients at PCF Nov. 2022
2023: Monumental Year Ahead
Accomplishments in 2022: Strong Foundation for Future Growth

Commercial and Clinical Execution

✓ Received FDA approval and launched IGALMI™

✓ Advanced 3 pivotal programs:
  • Progressed TRANQUILITY II Trial
  • Initiated TRANQUILITY III Phase 3 Trial
  • Initiated SERENITY III Phase 3 Trial

✓ Established OnkosXcel Therapeutics as independent entity
  • Presented positive data from Phase 2 trial of BXCL701

✓ Secured $260 million strategic financing
  • Well funded to advance toward key milestones
Thank you!