



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 and Important Safety Information

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](#).

Building a Unique Biopharmaceutical Business Model

Transformative Drug Re-innovation Approach Using AI

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*

OnkosXcel
Therapeutics
Advanced commercial
launch activities and clinical
pipeline development

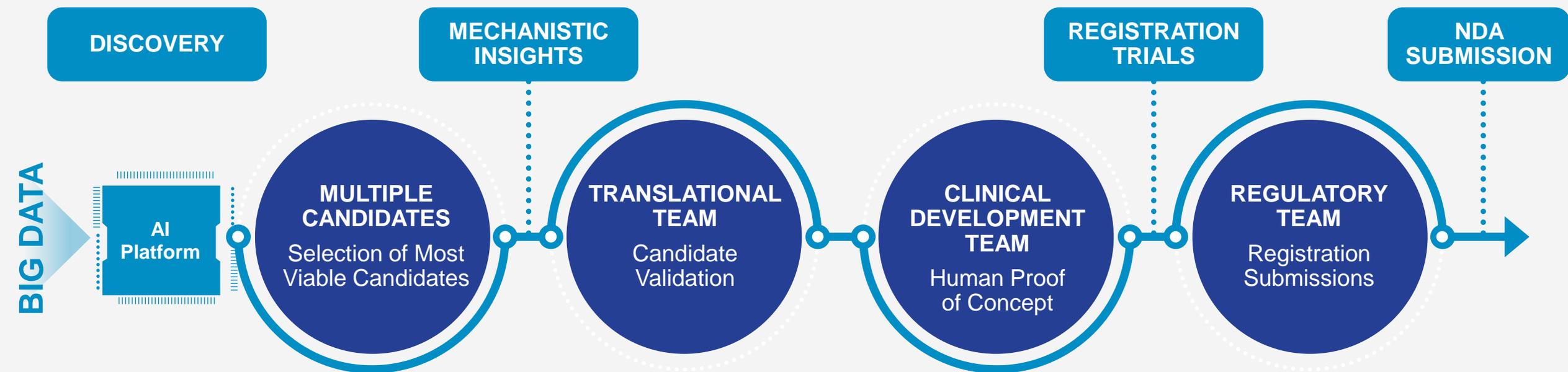


*Poised to
potentially
capture 139-
million-episode¹
U.S. agitation
market*

Bipolar disorders,
schizophrenia
& Alzheimer's-
related agitation

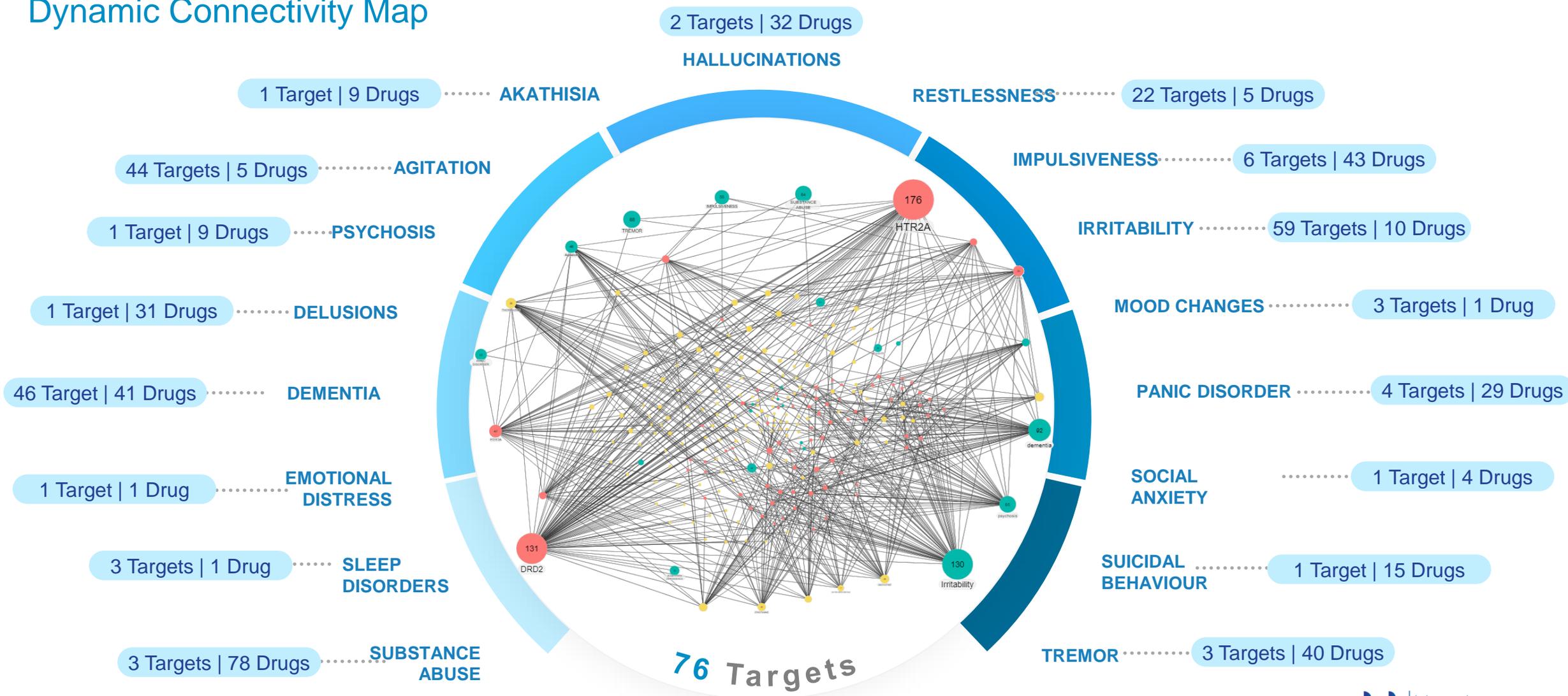
Uniquely Integrated Drug Discovery & Development Capability

Utilizing Proprietary AI Platform



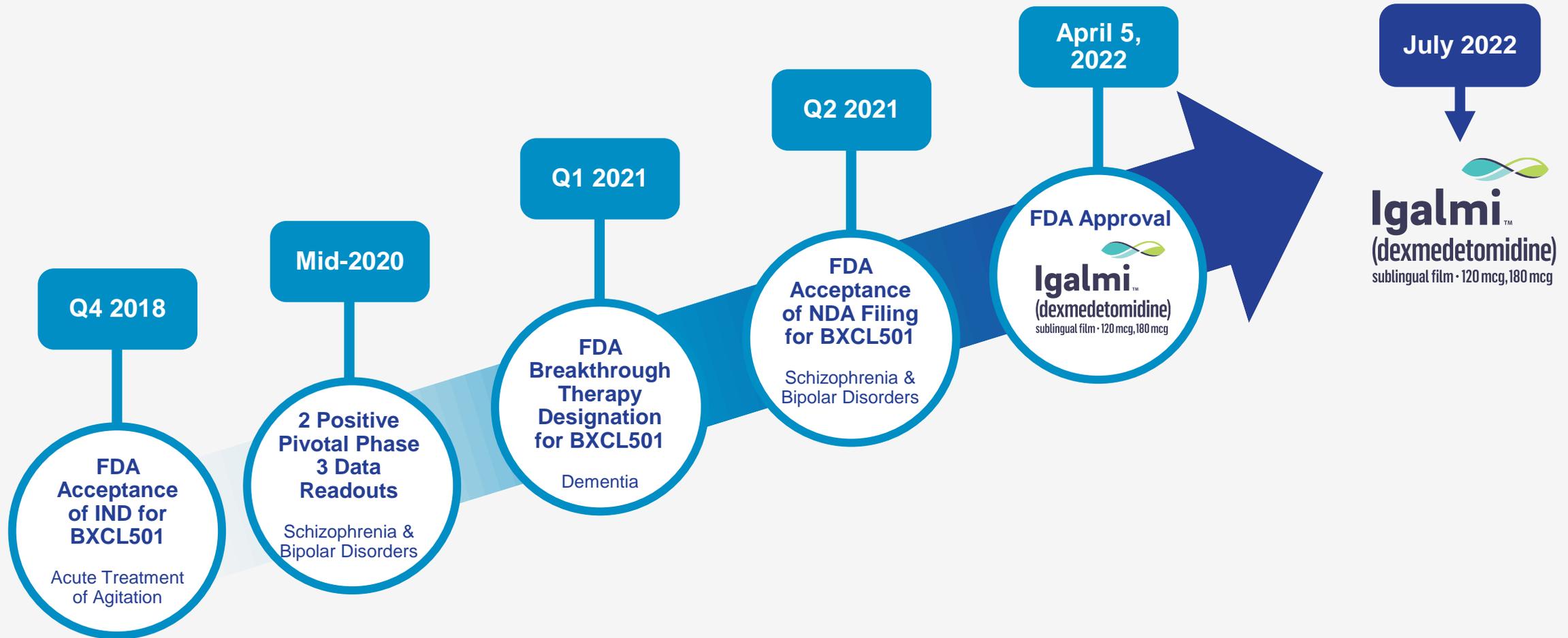
AI-Driven Insights Into Universe of Stress-related Symptoms, Targets & Drugs

Dynamic Connectivity Map



IND to IGALMI™ Launch in 4 Years: Proven Business Model

First AI-Derived, FDA-Approved Drug With Novel Mechanism of Action



Innovation Shaping the Agitation Market

Similar to Depression Market in the 1980s

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 world-wide anti-depressant market could expand faster than its current annual rate of about 10%.” (1992)*

Innovation
driving market
creation

Historically
**underdiagnosed
and underserved**
markets

**No commercial
precedent or
analogs**

IGALMI marketing and education
efforts are highlighting the
undervalued agitation market



IgalmiTM
(dexmedetomidine)
sublingual film • 120 mcg, 180 mcg

Well-Positioned to Help Address Significant U.S. Market Opportunity

*\$15B Potential Market Opportunity¹



16M
BPD/SCZ
Institutional
Episodes¹⁻³



IgalmiTM
(dexmedetomidine)
sublingual film • 120 mcg, 180 mcg

139M
Agitation Episodes¹⁻³

23M
BPD/SCZ
At-Home*
Episodes¹⁻³

BXCL501*
SERENITY III Pivotal Trial:
Top-line Data Readout Expected in 1H 2023

100M
Alzheimer's*
Episodes¹

BXCL501*
TRANQUILITY II
Top-line Data Readout
Expected in 1H 2023

1. Data on file. BioXcel Therapeutics, Inc. New Haven, CT December 2020. 2. Wu EQ, Shi L, Birnbaum H, et al. Annual prevalence of diagnosed schizophrenia in the USA: a claims data analysis approach. Psychol Med. 2006;36(11):1535-1540. 3. National Institute of Mental Health. Prevalence of bipolar disorder in adults. November 2017. Accessed December 16, 2022. <https://www.nimh.nih.gov/health/statistics/bipolar-disorder>.

*139M episodes @ \$105/episode *Investigational use; safety and efficacy not established

Potential Market-Changing Product & Current Pipeline

| Compound | Indication/Proposed Indication | Preclinical | Phase 1 | Phase 2 | Phase 3 | Registration | Marketed |
|---|--|------------------------------|---------|---------|---------|--------------|----------|
| Neuroscience | | | | | | | |
|  Igalmi (dexmedetomidine) <small>sublingual film - 120 mcg, 180 mcg</small> | Acute treatment of agitation associated with schizophrenia or bipolar I or II disorder in adults | Approved April 5, 2022 | | | | | |
| BXCL501 | At-home acute treatment of agitation associated with schizophrenia or bipolar I or II disorder in adults | SERENITY III | | | | | |
| | Acute treatment of agitation associated with Alzheimer's disease* | TRANQUILITY II & III | | | | | |
| | Adjunctive treatment in major depressive disorder | | | | | | |
| BXCL502 | Chronic agitation in Alzheimer's disease | | | | | | |
| Wearable Device (+BXCL501)** | Pre & post-agitation in dementia | Phase 0 device testing | | | | | |
| Immuno-oncology | | | | | | | |
|  | | | | | | | |
| BXCL701 | Small cell neuroendocrine metastatic castration-resistant prostate cancer | (Combination with KEYTRUDA®) | | | | | |

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

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

26
Reps

No field
account
team

Limited
Marketing

Initial
Speaker
Programs

No Free
Trial
Program

Building
Data
Lake



70
Reps

Corporate
Account
Director Team
Onboard

Enhanced
Advertising
to Raise
Awareness

Amplified
Speaker
Programs
in H1

Free
Trial
Program

Leveraging
Data Lake

2023

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



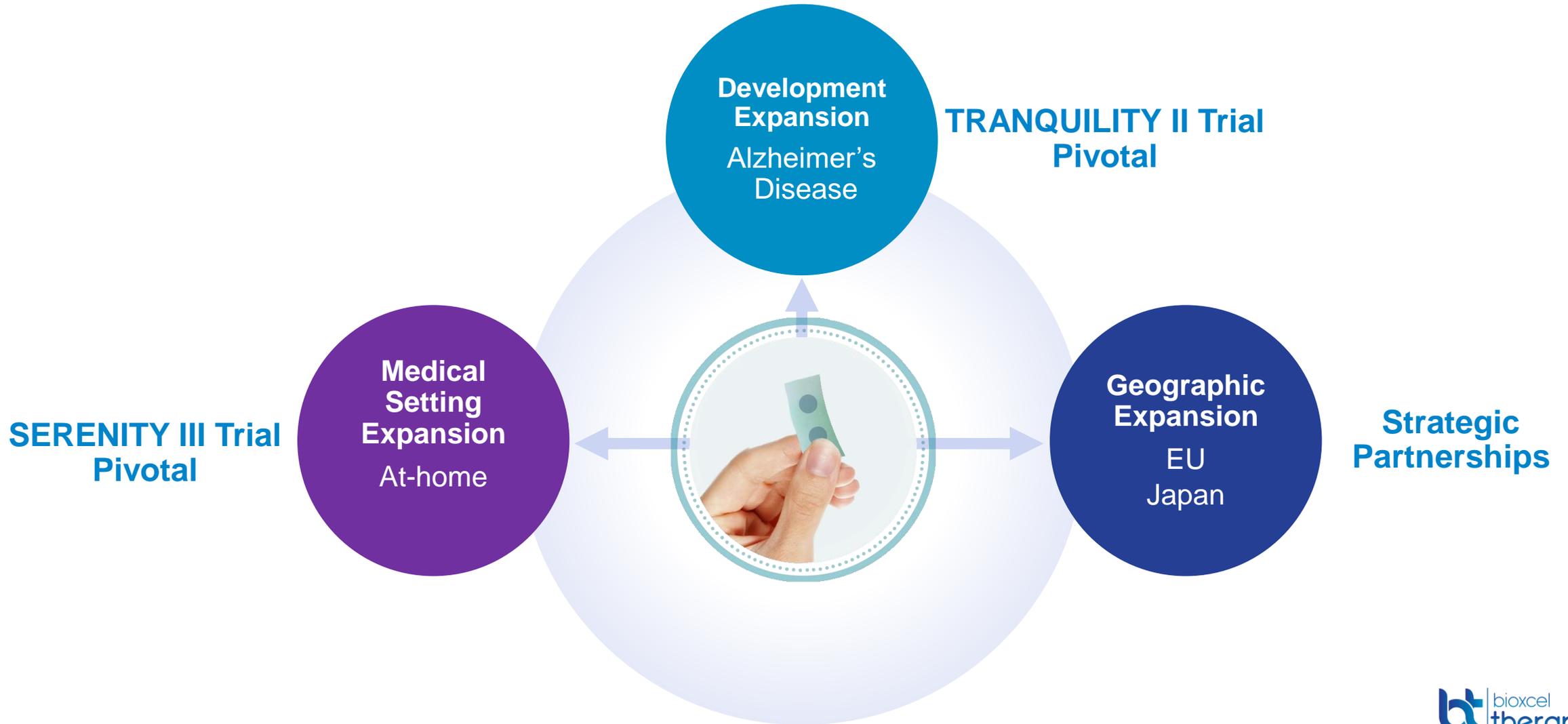
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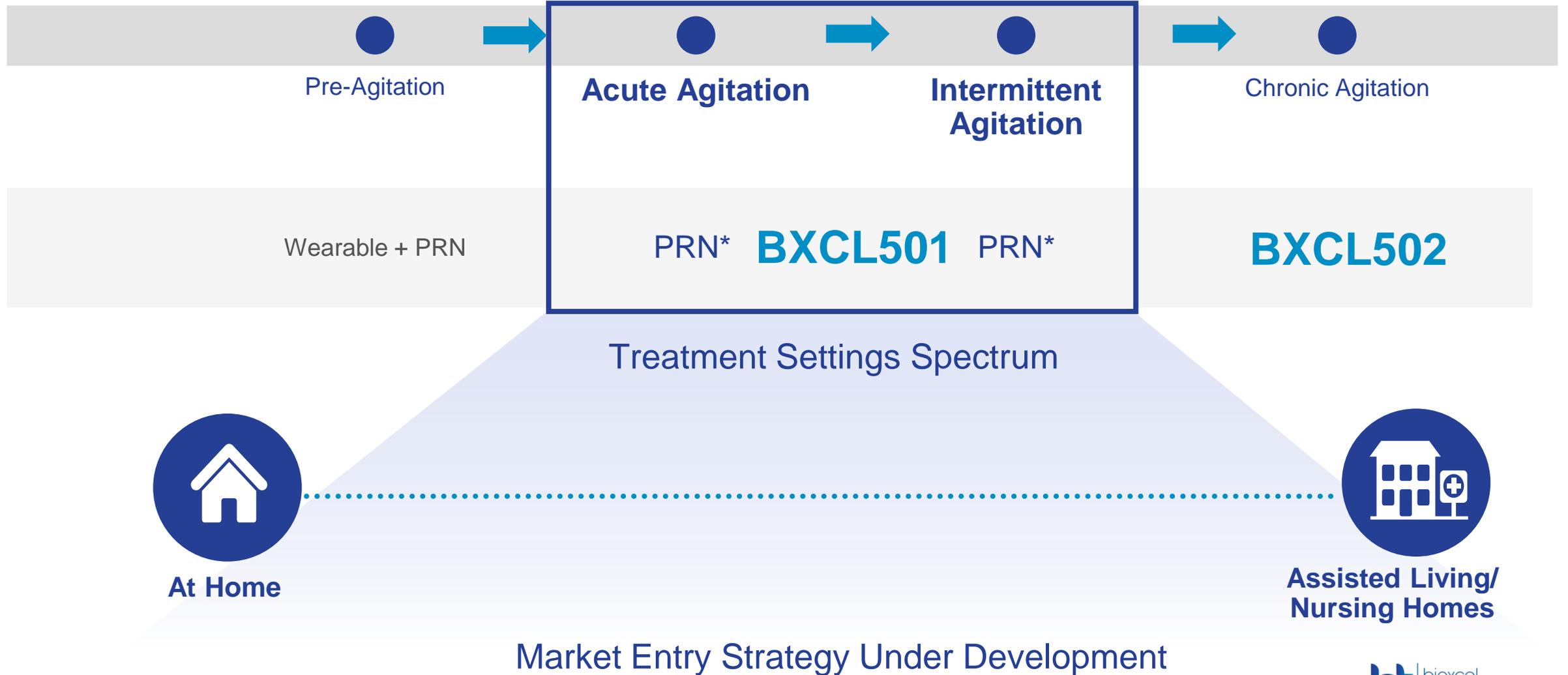
Alzheimer's Related Agitation

Breakthrough Therapy Designation

Our Land and Expand Strategy

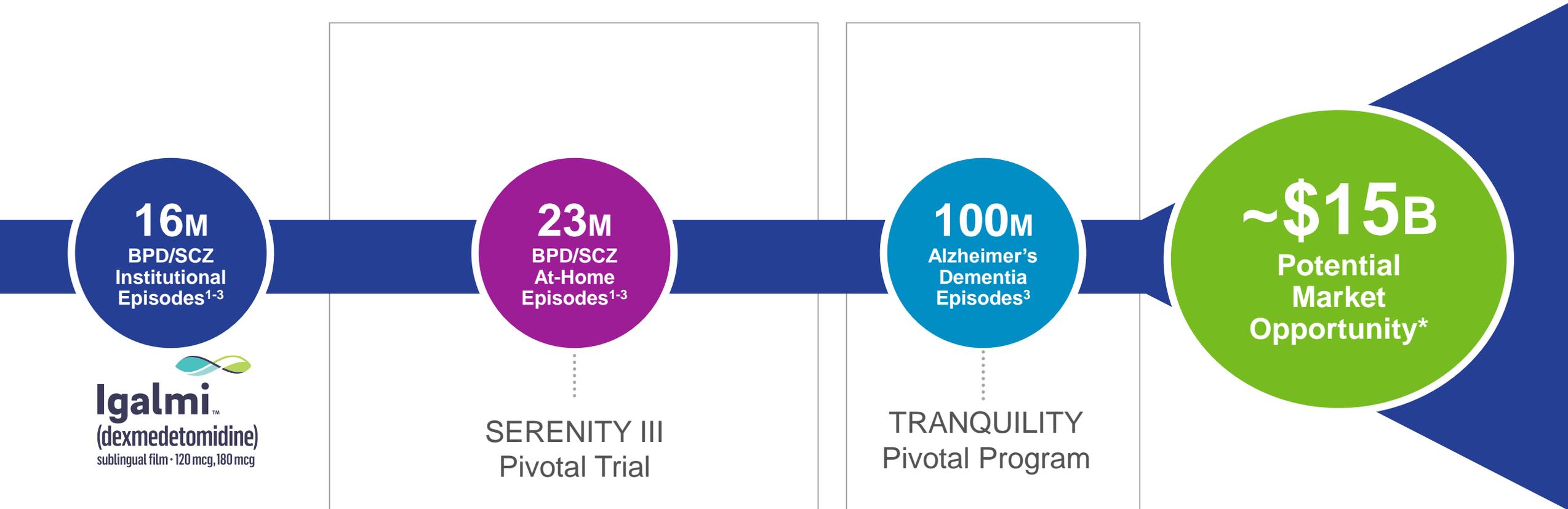


Alzheimer's Program: Addressing Entire Agitation Spectrum



Significant Market Opportunity: At-home & Alzheimer's Agitation

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



1. Wu EQ, Shi L, Birnbaum H, et al. Annual prevalence of diagnosed schizophrenia in the USA: a claims data analysis approach. Psychol Med. 2006;36(11):1535-1540 2. National Institute of Mental Health. Prevalence of bipolar disorder in adults. November 2017. Accessed December 16, 2022. <https://www.nimh.nih.gov/health/statistics/bipolar-disorder> 24, 2021. 3. Data on file. BioXcel Therapeutics, Inc. New Haven, CT December 2020
*139M episodes @ \$105/episode

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

Distinct Leadership in Innate Immunity DPP8/9 Biology

Mechanism of Action Published in JITC

One of most advanced oral innate immune activators activates inflammasome via DPP8/9 inhibition

Distinct Position in Innate Immunity DPP8/9 Biology



Scarcity of assets in innate immunity



Clinical Proof of Concept Cold Tumors

Demonstrated efficacy in rare cancer: small cell neuroendocrine prostate cancer (SCNC)

Full Data Set for SCNC at ASCO GU

Response rate: 33%*
Mean duration of response: 9 months
Generally well tolerated in combination with KEYTRUDA®

*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™


Igalmi™
 (dexmedetomidine)
 sublingual film • 120 mcg, 180 mcg



- ✓ 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

OnkosXcel
Therapeutics™
A subsidiary of Biocel Therapeutics, Inc.



- ✓ Secured \$260 million strategic financing

- Well funded to advance toward key milestones

Thank you!

