



# AI-Driven Transformative Medicines in Neuroscience

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April 2024

# Forward-Looking Statements

This presentation includes “forward-looking statements” within the meaning of the Private Securities Litigation Reform Act of 1995. BioXcel Therapeutics, Inc. (“BioXcel” or the “Company”) intends such forward-looking statements to be covered by the safe harbor provisions for forward-looking statements contained in Section 27A of the Securities Act of 1933, as amended and Section 21E of the Securities Exchange Act of 1934, as amended. All statements contained in this presentation other than statements of historical fact should be considered forward-looking statements, including, without limitation, statements related to the safety, efficacy, and regulatory and clinical design or progress, potential regulatory submissions, approvals and timing thereof for BXCL501 as a potential acute treatment for AAD or for agitation associated with bipolar disorders or schizophrenia in the at-home setting; developments and plans relating to the SERENITY and TRANQUILITY programs; and the potential for the results from the Company’s completed, ongoing and proposed clinical trials to support regulatory approvals for its product candidates in both the care-facility and at-home settings. When used herein, words including “anticipate,” “believe,” “can,” “continue,” “could,” “designed,” “estimate,” “expect,” “forecast,” “goal,” “intend,” “may,” “might,” “plan,” “possible,” “potential,” “predict,” “project,” “should,” “target,” “will,” “would” and similar expressions are intended to identify forward-looking statements, though not all forward-looking statements use these words or expressions. 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 the Company’s current expectations and various assumptions. The Company believes there is a reasonable basis for its expectations and beliefs, but they are inherently uncertain.

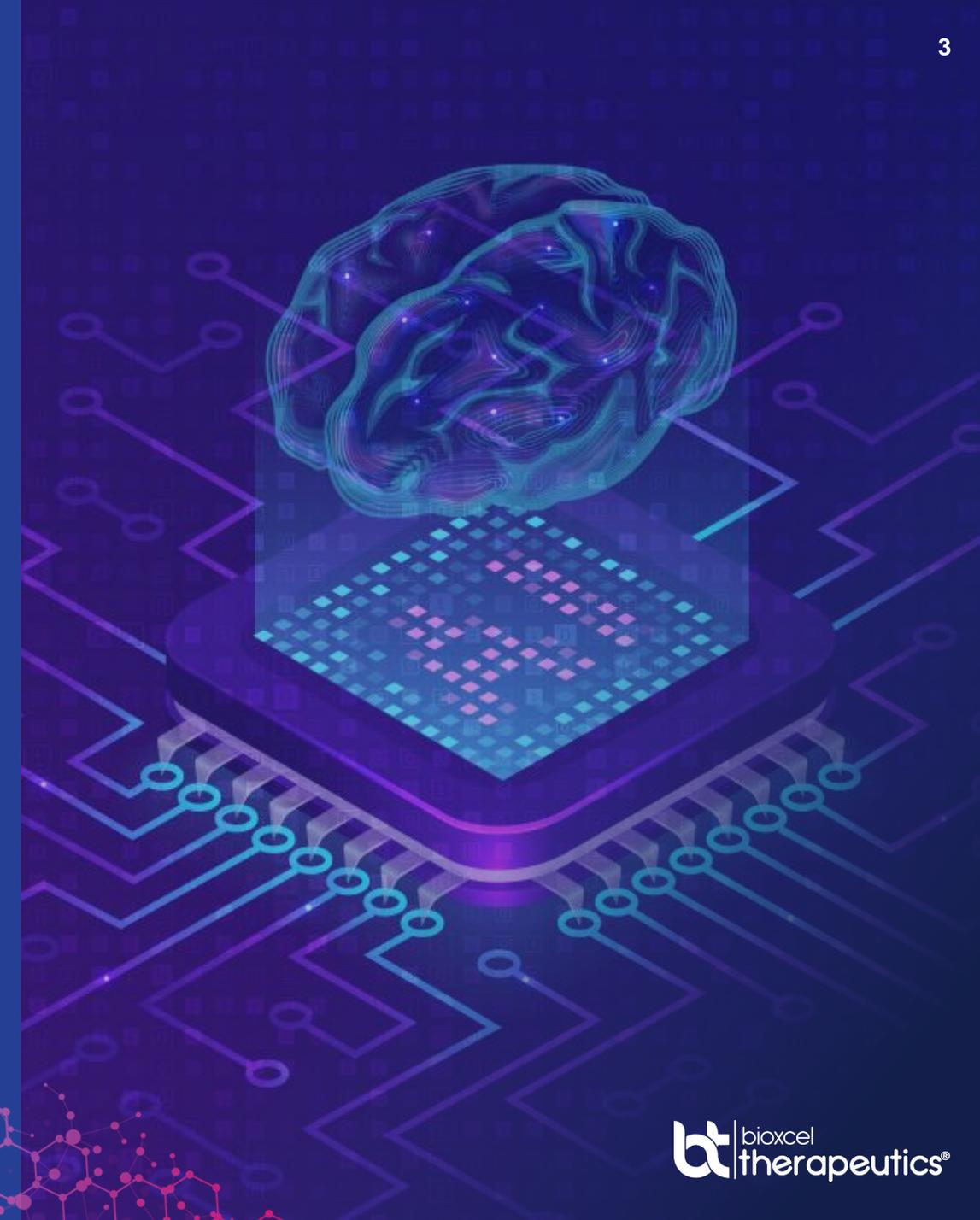
The Company may not realize its expectations, and its beliefs may not prove correct. Actual results could differ materially from those described or implied by such forward-looking statements as a result of various important factors, including, without limitation: its limited operating history and limited revenue generation; its incurrence of significant losses; its strategic reprioritization and related reduction in force may not achieve its intended outcome; its need for substantial additional funding and ability to raise capital when needed; its significant indebtedness, ability to comply with covenant obligations and potential payment obligations related to such indebtedness and other contractual obligations; the Company has identified conditions and events that raise substantial doubt about its ability to continue as a going concern; its limited experience in drug discovery and drug development; risks related to the TRANQUILITY program; risks related to the limited clinical data supporting potential safety or efficacy of BXCL501 for use in the at-home setting; its dependence on the success and commercialization of IGALMI, BXCL501, BXCL502, BXCL701 and BXCL702 and other product candidates; interim “top-line” and preliminary data from its clinical trials may change and result in material changes in the final data; its ability to receive regulatory approval from the FDA and comparable foreign authorities for its product candidates; clinical trials are expensive, time-consuming, difficult to design, difficult to conduct, and involve an uncertain income; its lack of experience in marketing and selling drug products; the risk that IGALMI or the Company’s product candidates may not be accepted by physicians or the medical community in general; the Company’s estimated number of episodes of agitation and its corresponding estimated total addressable market are subject to inherent challenges and uncertainties; the Company still faces extensive and ongoing regulatory requirements and obligations for IGALMI; 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 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; the significant influence of and dependence on BioXcel LLC; its exposure to patent infringement lawsuits; its reliance on third parties; its ability to comply with the extensive regulations applicable to it; impacts from data breaches or cyber-attacks, if any; the Company is and may in the future be subject to legal proceedings, claims and investigations in or outside the ordinary course of business, which could be costly and time-consuming to defend and could result in unfavorable outcomes; risks related to unfavorable global political or economic events and conditions; risks associated with the increased scrutiny relating to environmental, social and governance (ESG) matters; risks associated with federal, state or foreign health care “fraud and abuse” laws; and its ability to commercialize its product candidates, as well as the important factors discussed under the caption “Risk Factors” in its Annual Report on Form 10-K for the fiscal year ended December 31, 2023, which are accessible on the SEC’s website at [www.sec.gov](http://www.sec.gov) and the Investors section of the Company’s website at [www.bioxceltherapeutics.com](http://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 the Company 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 the Company’s views to change. These forward-looking statements should not be relied upon as representing the Company’s views as of any date subsequent to the date of this presentation.

## INDUSTRY AND OTHER DATA

Unless otherwise indicated, information contained in this presentation concerning our industry and the markets in which BioXcel Therapeutics operates, including its general expectations, market position and market opportunity, is based on its management’s estimates and research, as well as industry and general publications and research, surveys and studies conducted by third parties. While BioXcel Therapeutics believes the information from these third-party publications, research, surveys and studies is reliable, it does not guarantee the accuracy or completeness of such information, and BioXcel Therapeutics has not independently verified this information. Management’s estimates are derived from publicly available information, their knowledge of the company’s industry and their assumptions based on such information and knowledge, which they believe to be reasonable. This data involves a number of assumptions and limitations which are necessarily subject to a high degree of uncertainty and risk due to a variety of factors, including those described in BioXcel Therapeutics’ periodic reports filed with the SEC under the captions “Forward Looking Statements,” “Risk Factor Summary” and “Risk Factors.” These and other factors could cause BioXcel Therapeutics’ future performance and market expectations to differ materially from its assumptions and estimates.

# Corporate Overview

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# About BioXcel Therapeutics



**Founded:** 2017

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**IPO:** 2018

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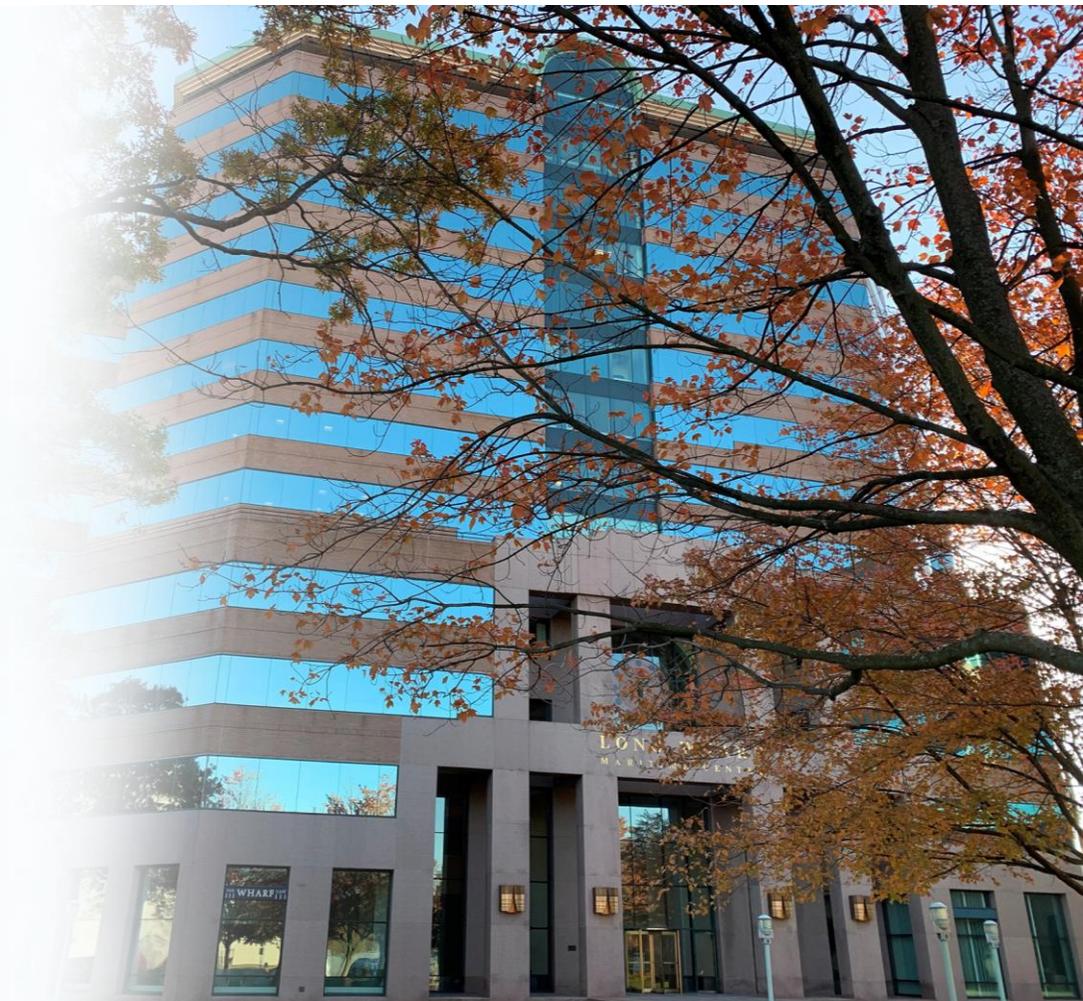
**Ticker:** BTAI (Nasdaq)

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**Headquarters:** New Haven, CT

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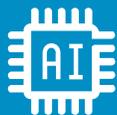


# Our Mission

Develop transformative  
medicines in neuroscience  
utilizing artificial intelligence

# Strong Value Proposition and Long-Term Growth Potential

Transformative approach leveraging technology, clinical, and commercial expertise



## Unique, Proven Business Model

- Clinically and commercially validated AI platform
- BXCL501 IND to IGALMI™ approval < 4 years



## 2 Advanced Phase 3 Programs

- **TRANQUILITY**: agitation associated with Alzheimer's dementia (AAD)
- **SERENITY**: agitation associated with bipolar disorders or schizophrenia (at-home setting)



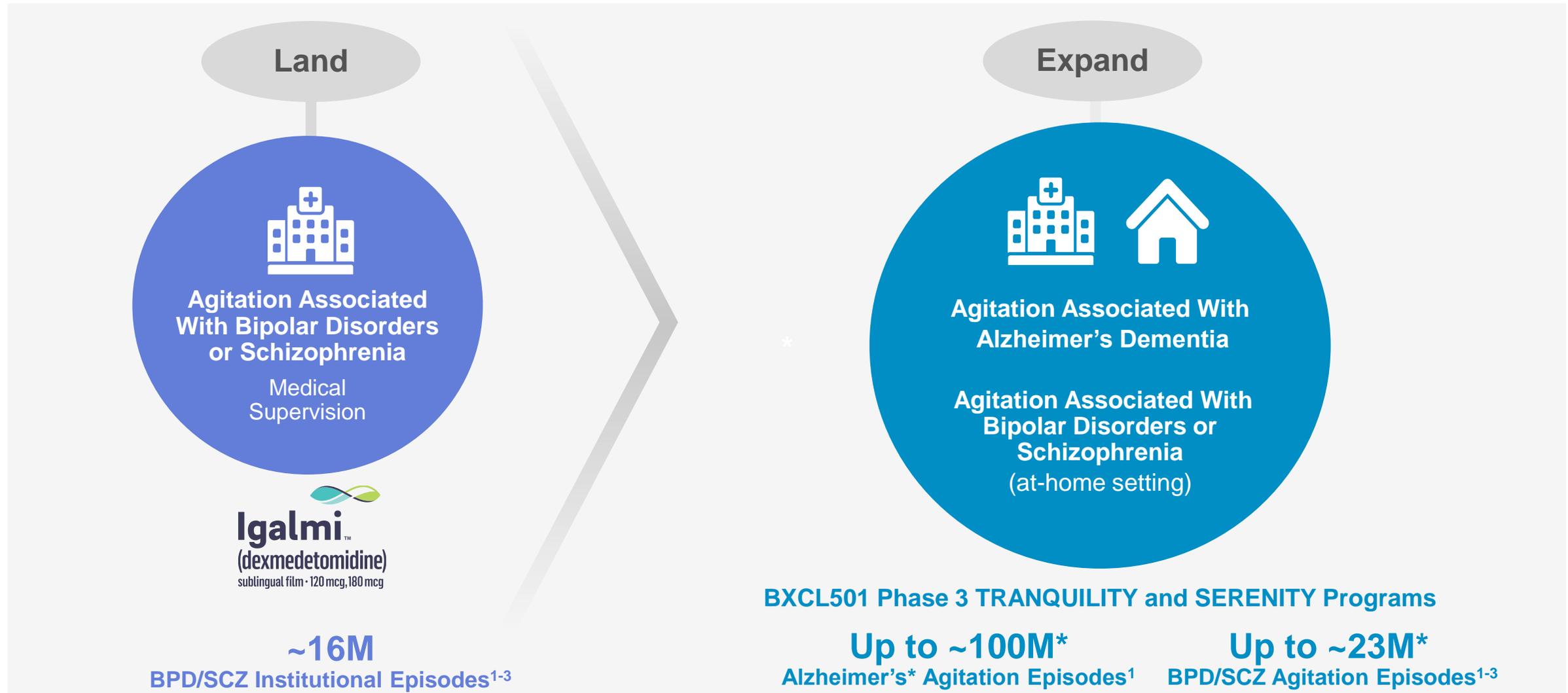
## Large U.S. Agitation Market Opportunity\*

- **Up to 7M** patients with Alzheimer's in U.S.; 50% have agitation (~100M AAD episodes<sup>1</sup>)
- **Up to ~23M** BPD/SCZ agitation episodes in at-home setting<sup>1-3</sup>

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>.

\*Actual addressable market may be smaller

# Corporate Growth Drivers to Advance Land and Expand Strategy



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

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>. \*Actual addressable market may be smaller

# Neuroscience Clinical Development

Compound	Indication/Proposed Indication	Preclinical	Phase 1	Phase 2	Phase 3	Registration	Marketed
<b>IGALMI™</b> (dexmedetomidine) sublingual film	Acute treatment of agitation associated with schizophrenia or bipolar I or II disorder in adults under healthcare provider supervision						
<b>BXCL501</b>	<u>TRANQUILITY PROGRAM</u> Acute treatment of agitation associated with Alzheimer's dementia						
	<u>SERENITY PROGRAM</u> Acute treatment of agitation associated with bipolar disorders/schizophrenia (at home)						
	Opioid Use Disorder (OUD)*						
	Post Traumatic Stress Disorder (PTSD)*						
<b>BXCL502</b> (latrepirdine)	Chronic agitation in dementia						
<b>Candidate BXCL503</b>	Apathy in dementia						
<b>Candidate BXCL504</b>	Aggression in dementia						

\*Government-funded, investigator-sponsored trials

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

As of April 22, 2024

# Leadership Expertise



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

**Richard I. Steinhart**  
Senior Vice President & Chief Financial Officer

**Frank D. Yocca, Ph.D.**  
Senior Vice President & Chief Scientific Officer

**Vincent J. O'Neill, M.D.**  
Executive Vice President, Chief of Product Development and Medical Officer

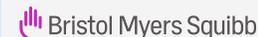
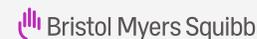
**Javier Rodriguez**  
Senior Vice President, Chief Legal Officer, and Corporate Secretary

**Matt Wiley**  
Senior Vice President & Chief Commercial Officer

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

**Chetan D. Lathia, Ph.D.**  
Senior Vice President & Head of Regulatory Affairs

**Dusan Kostic, Ph.D.**  
Vice President, Head of Medical Affairs



# Acute Treatment of Agitation Associated with Alzheimer's Dementia (AAD)

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TRANQUILITY Program



# AAD is Debilitating for Patients and a Burden for Caregivers

Agitation cited as a top driver in deciding to move a patient from home setting to residential care facility<sup>1</sup>

- Nearly 7 million Alzheimer's dementia patients in the U.S., with approximately 50% suffering from agitation.<sup>2</sup>
- AD-related agitation typically worsens over time<sup>2</sup>
  - Both the number and severity of agitation episodes increase<sup>2</sup>
  - Often places significant burden on caregivers<sup>1,2</sup>
- No FDA-approved therapeutic options for an as-needed (PRN) acute treatment of agitation in Alzheimer's patients<sup>3</sup>



1. Data on File InVibe Patient and Caregiver Research (n=75) December 2022

2. Alzheimer's Association. 2023 Alzheimer's Disease Facts and Figures. Accessed November 14, 2023. <https://www.alz.org/media/Documents/alzheimers-facts-and-figures.pdf>;  
Halpern R, Seare J, et al. Using electronic health records to estimate the prevalence of agitation in Alzheimer disease/dementia. Int J Geriatr Psychiatry. 2019; 34: 420-431.

3. Joint Meeting of the Psychopharmacologic and the Peripheral and Central Nervous System Drugs Advisory Committee Meeting April 14<sup>th</sup>, 2023

# TRANQUILITY Program Offers Potential Path to sNDA



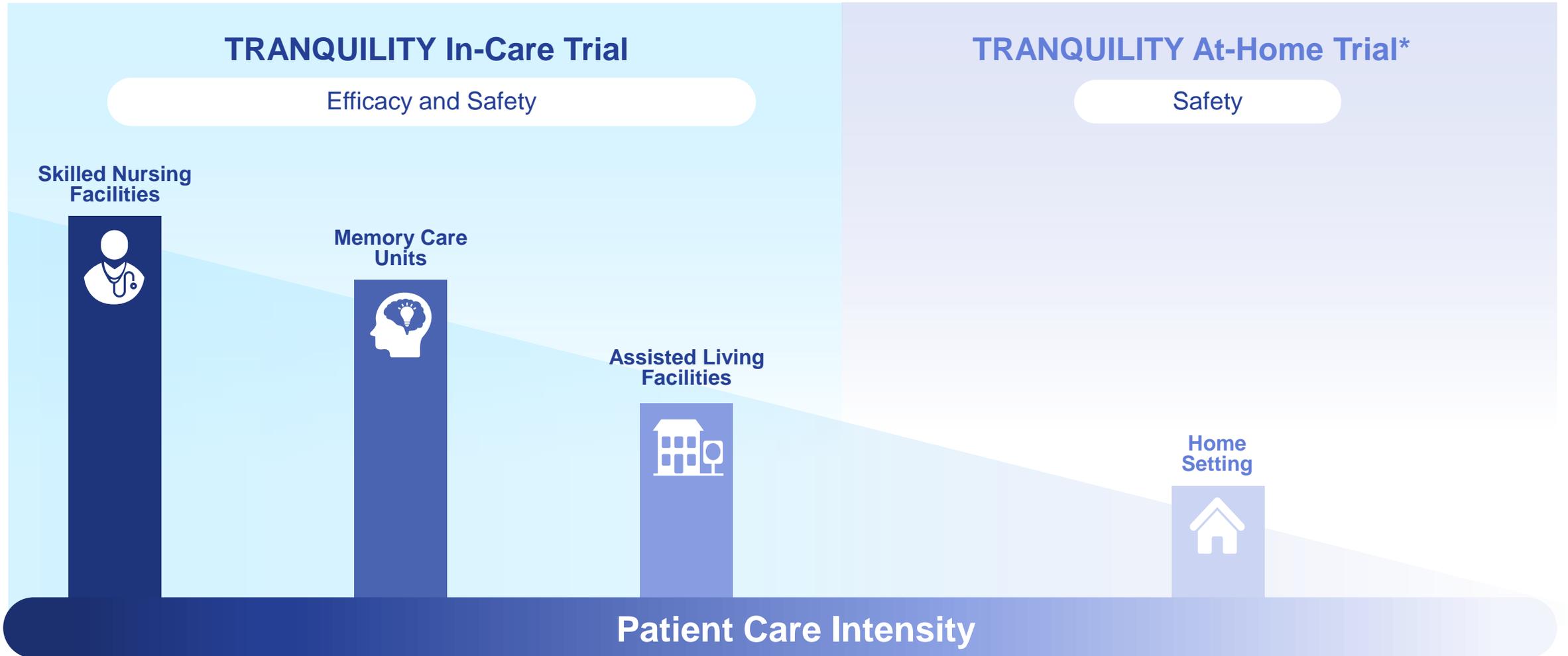
- Plan to discuss details of requirement for long-term safety data at future meeting with FDA\*\*
- Company has developed preliminary TRANQUILITY At-Home trial design and is re-evaluating initiation timing

\* Trial protocol under development, design may be subject to change.

\*\* Per ICH guidelines, the Company may be required to collect 6-month safety data from at least 300 patients and 1-year safety data from at least 100 patients prior to submitting any sNDA

# Evaluating BXCL501 for AAD in High to Low Care Settings

Clinical trial strategy designed to maximize potential commercial opportunity across patient locations



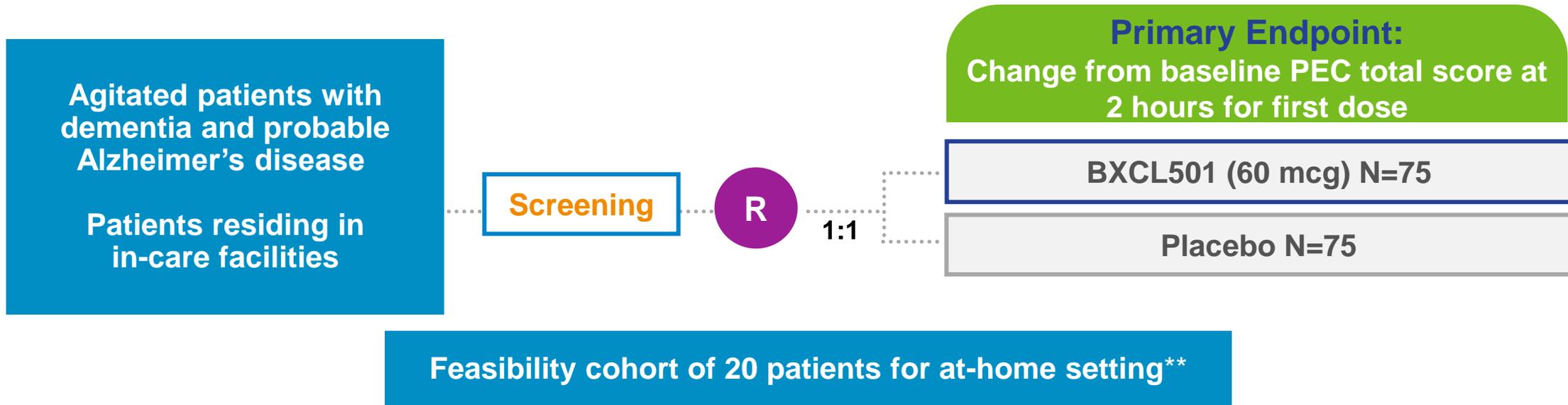
\*Trial design may be subject to change

**Skilled-Nursing Facilities:** medical setting for patients with advanced health conditions who receive 24/7 skilled nursing care and medical monitoring

**Memory Care Units:** medical setting for patients with dementia who receive specialized care for symptom management

**Assisted Living Facilities:** residential setting for elderly patients who are largely independent but need help with ADLs (bathing, dressing, and other non-medical type assistance)

# TRANQUILITY In-Care Study Design\*

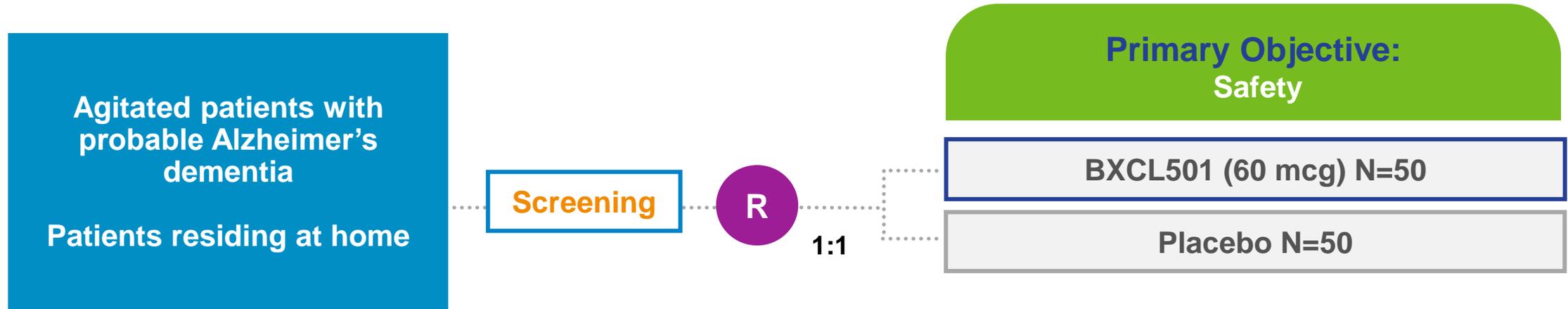


- **Design:** Randomized, double-blind, placebo-controlled, parallel group trial
- **Power:** Over 80% power
- **Inclusion Criteria**
  - Patients with probable AD (mild, moderate, or severe, MMSE  $\leq$  25), who experience agitation, and residing in skilled nursing facilities, memory care units, or assisted living facilities
  - Patients with episodes of agitation in the month prior to enrollment
  - PEC total score  $\geq$ 14 prior to randomization
- **Primary Endpoint:** Change from baseline of PEC total score at 2 hours for **first dose**
- **Study Duration:** 12 weeks with assessment of continued efficacy (up to 3 PECs)

\*For illustrative purposes only: protocol under development and trial design may be subject to change. The FDA has not provided feedback on this trial.

\*\* Represents a separate cohort of 20 patients who reside at home in addition to the 150 patients who are in care facilities

# Preliminary TRANQUILITY At-Home Study Design\*



- **Study Design:** Randomized, double-blind, placebo-controlled, parallel group trial
- **Primary Objective:** Safety and tolerability of BXCL501 60 mcg
- **Inclusion Criteria**
  - Patients with mild, moderate, or severe probable AD who experience agitation, MMSE  $\leq$  25
  - Patients with **not more than three episodes of agitation per week** in the month prior to enrollment
  - Patients with caregivers
- **Treatment**
  - BXCL501 60 mcg or placebo administered for agitation in at-home setting

\* For illustrative purposes only. Protocol under development and trial design may be subject to change. The FDA has not provided feedback on this trial.

# Acute Treatment of Agitation Associated with Bipolar Disorders or Schizophrenia (*at-home setting*)

SERENITY Program



# Agitation: Relatively Common and Difficult-to-Manage<sup>1</sup>

## Debilitating for Patients and Threatening for Healthcare Providers



Characterized by recurring episodes<sup>1</sup>



Symptoms differ by patient, vary between episodes, and range from mild to severe<sup>2-7</sup>



Multi-billion-dollar healthcare burden<sup>8</sup>



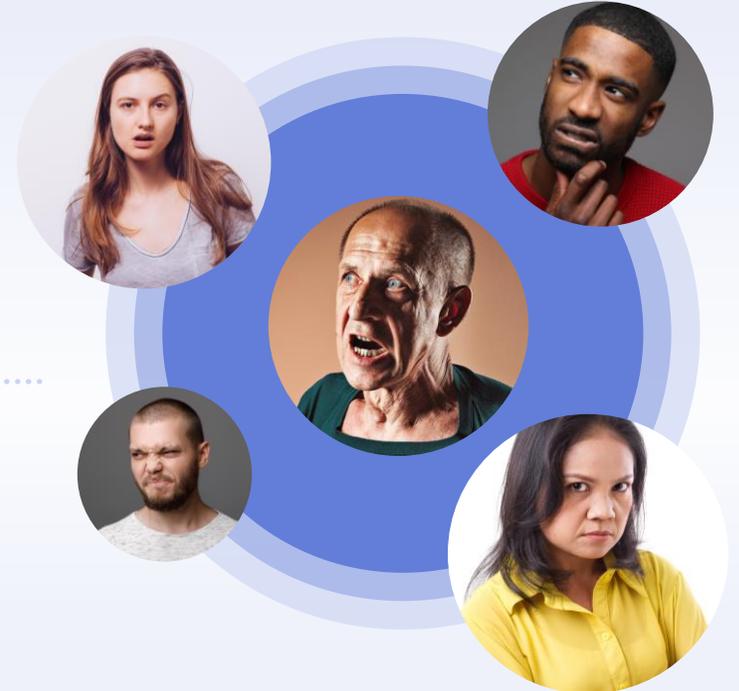
Best-practice guidelines recommend agitation be treated by:

- Behavioral calming techniques
- Verbal de-escalation
- Medications voluntarily accepted by patients without coercion; pharmacologic goal of calming without unarousable sedation<sup>9</sup>



Current treatment approaches:

- May involve physically restraining patients<sup>10</sup>
- Over-sedating therapies such as antipsychotics and benzodiazepines<sup>10</sup>



1. Sacchetti E, Amore M, Di Sciascio G, et al. Psychomotor agitation in psychiatry: an Italian expert consensus. *Evidence-based Psychiatric Care*. 2017;1:1-24. 2. Dunder Y, Greenhalgh J, Richardson M, et al. Pharmacological treatment of acute agitation associated with psychotic and bipolar disorder: a systematic review and meta-analysis. *Hum. Psychopharmacol*. 2016;31(4):268-285. 3. Garriga M, Pacchiarotti I, Kasper S, et al. Assessment and management of agitation in psychiatry: expert consensus. *World J Biol Psychiatry*. 2016;17(2):86-128. 4. Nordstrom K, Zun LS, Wilson MP, et al. Medical evaluation and triage of the agitated patient: consensus statement of the American association for emergency psychiatry project Beta medical evaluation workgroup. *West J Emerg Med*. 2012;13(1):3-10. 5. Martinez-Raga J, Amore M, Di Sciascio G, et al. 1st international experts' meeting on agitation: conclusions regarding the current and ideal management paradigm of agitation. *Front. Psychiatry*. 2018;9(54):1-9. 6. Depression and Bipolar Support Alliance (DBSA). Understanding agitation: recognizing the signs of agitation and knowing what to do when they appear. 2014. 7. Montoya A, Valladares A, Lizán L, et al. Validation of the excited component of the positive and negative syndrome scale (PANSS-EC) in a naturalistic sample of 278 patients with acute psychosis and agitation in a psychiatric emergency room. *Health Qual Life Outcomes*. 2011;9:18. 8. Cloutier M, Gauthier-Loiselle M, Gagnon-Sanschagrin P, Guerin A, Hartry A, Baker RA, Duffy R, Gwin K, Sanon Aigbogun M. Institutionalization risk and costs associated with agitation in Alzheimer's disease. *Alzheimers Dement (N Y)*. 2019 Nov 23;5:851-861. 9. Wilson MP, Pepper D, Currier GW, et al. The psychopharmacology of agitation: consensus statement of the American Association for Emergency Psychiatry Project Beta Psychopharmacology Workgroup. *West J Emerg Med*. 2012;13(1):26-34. 10. Zeeller SL, Citrome L. Managing agitation associated with schizophrenia and bipolar disorder in the emergency setting. *West J Emerg Med*. 2016;17(2):165-172. doi: 10.1016/j.trci.2019.10.004. PMID: 31799369; PMCID: PMC6881649.9

# SERENITY Program Seeks Potential IGALMI™ Label Expansion

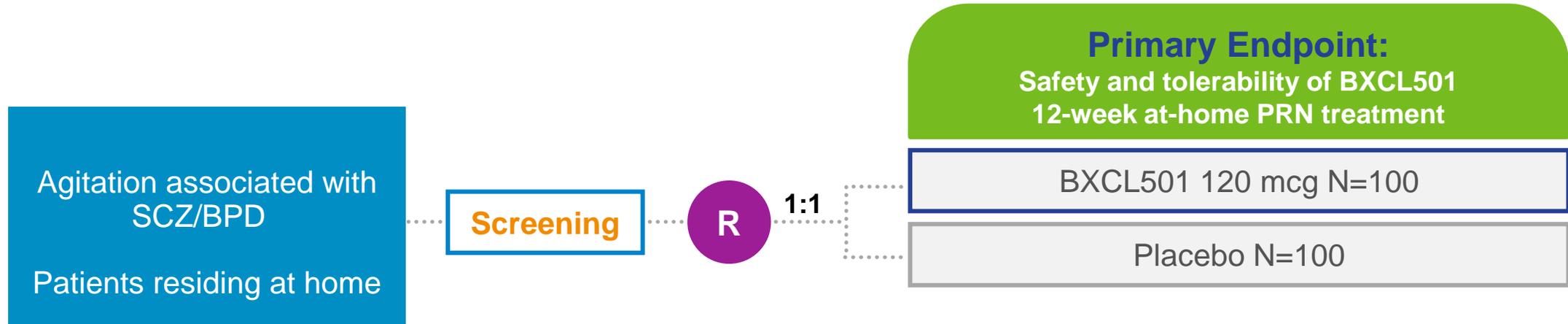
Potential opportunity for at-home use in treating agitation associated with bipolar disorders or schizophrenia



\* SERENITY At-Home represents the redesigned SERENITY III

† Trial protocol under development, design may be subject to change.

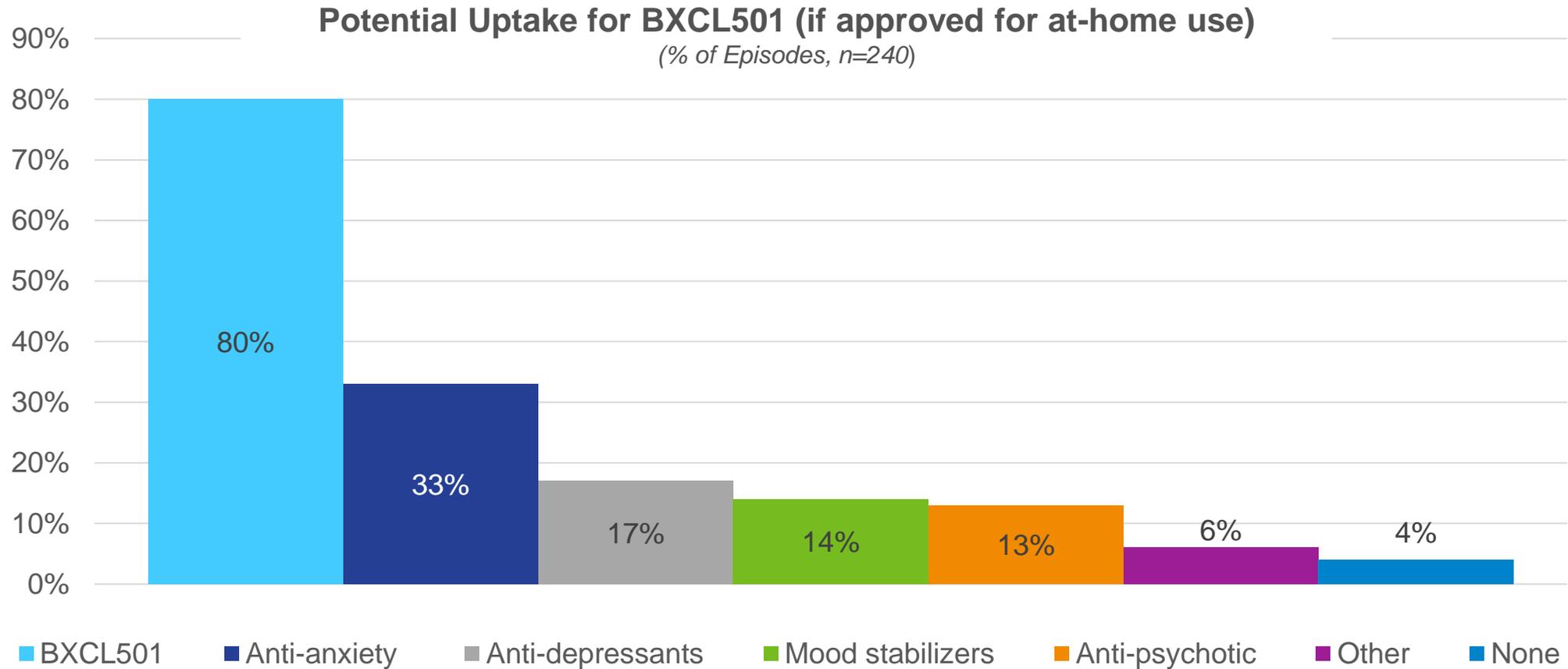
# Planned SERENITY At-Home Study Design



- **Recruitment Criteria**
  - Patients alone or with informants with at least 1 treated episode of agitation
  - Patients with episodes of agitation in the month prior to enrollment
- **Treatment**
  - Single dose to treat agitation at levels that typically require intervention
  - Maximum of 1 dose of study medication within 12 hours

# Potential for Bipolar Disorder/Schizophrenia Patient Use of BXCL501 At Home

When shown product profile stimulus, patients said they would use a product with the BXCL501 target profile for 80% of their bipolar/schizophrenia agitation episodes, and for those on therapy it could be adjunctive



Q22. You previously indicated that you used the following medications to manage your last 3 agitation episodes. Now please imagine that Igalmi was also available for you to use. Please indicate what treatment you would have chosen to treat the last 3 episodes if Igalmi were also available to you. We have provided your previous below for reference.

# IGALMI™ Commercialization

*Following commercial field workforce reduction in August 2023*

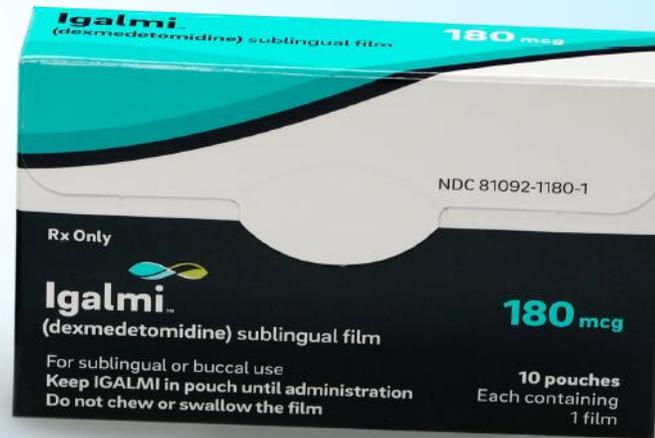
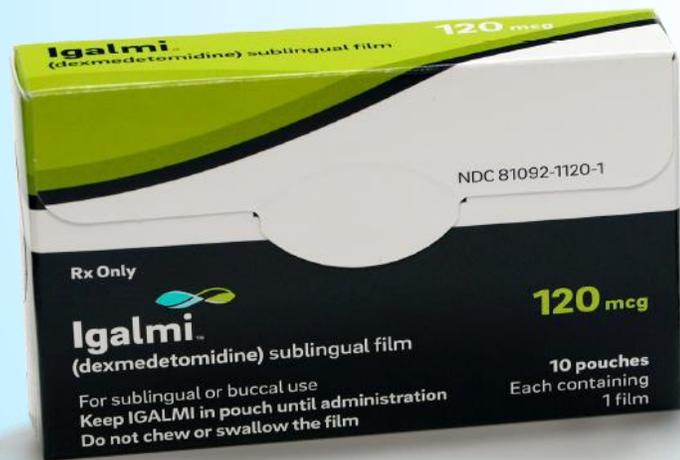


# IGALMI™ (dexmedetomidine) Sublingual Film

Approved for acute treatment of agitation associated with schizophrenia or bipolar I or II disorder in adults under healthcare provider supervision



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



## IGALMI™ 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.

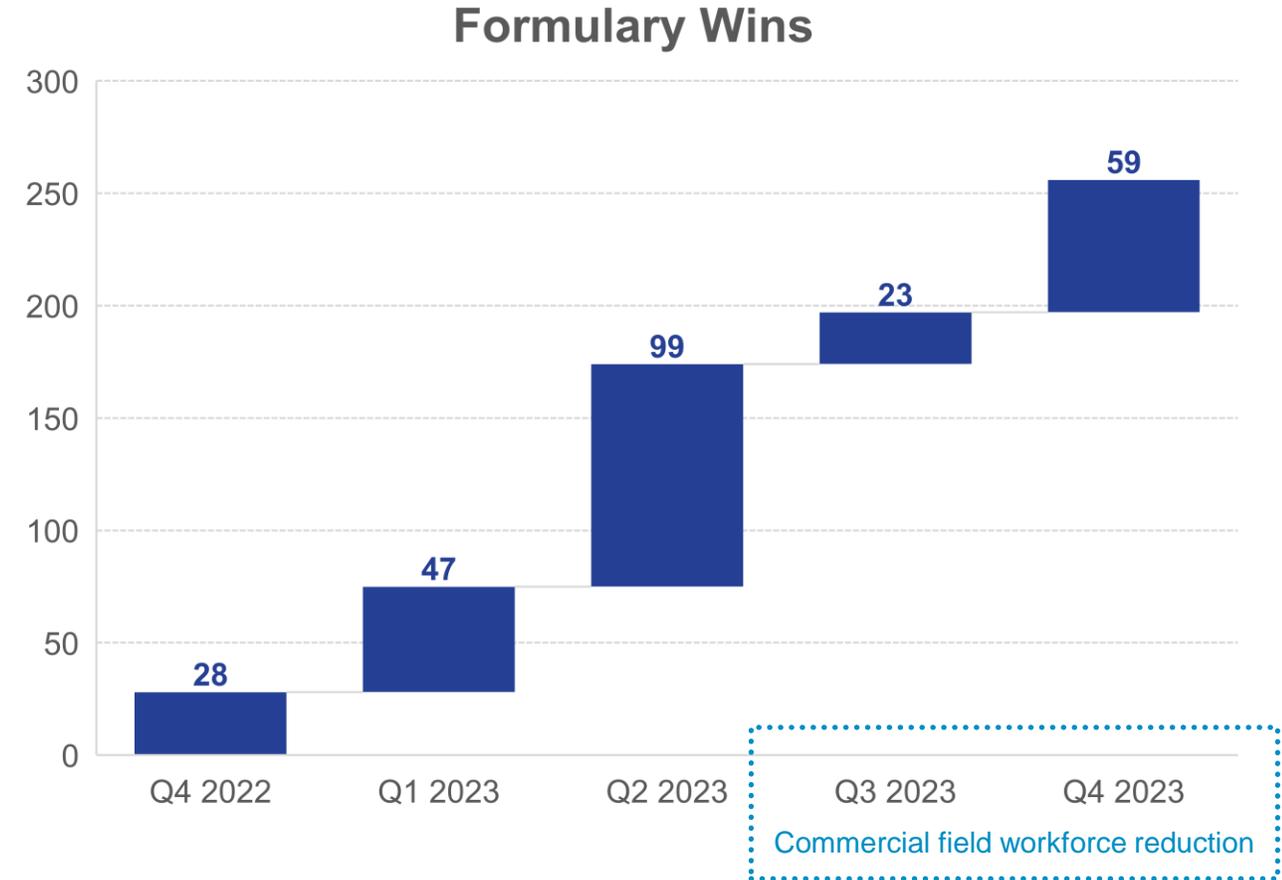
Everyone is encouraged to report negative side effects of prescription drugs to the FDA. Visit [www.fda.gov/medwatch](http://www.fda.gov/medwatch) or call 1-800-FDA-1088. You can also contact BioXcel Therapeutics, Inc. at 1-833-201-1088 or [medinfo@bioxceltherapeutics.com](mailto:medinfo@bioxceltherapeutics.com).

Please see full [Prescribing Information](#).

# Market Conditions Favorable for Continued Demand Growth

J-Code issuance, P&T formulary wins, and volume contracting to drive commercial progress following commercial field workforce reduction

- Targeting/engaged with key hospital systems for volume-based contracting
- J-Code granted by CMS effective Jan. 1, 2024 (streamlines and standardizes reimbursement)
- Over 250 hospital P&T approvals to date, with large volume in Q4 2023
- First large academic center approval in Q4 2023
- Activating new channels such as Dept. of Veterans Affairs and Dept. of Corrections



# Agitation Franchise Expansion Plan

Seek approvals for BXCL501 for agitation associated with bipolar disorder, schizophrenia, and Alzheimer's dementia in all settings



**Launched & established IGALMI™ in hospital setting for bipolar disorder/schizophrenia**

Use contracting and pricing to drive familiarity with IGALMI in hospitals

**Igalmi™**  
(dexmedetomidine)  
sublingual film - 120 mcg, 180 mcg

**Prepare for potential expansion into in-care and at-home markets for BXCL501**

Leverage patient and HCP experiences to prepare for launches if approved

**Potential launch for approved indications for BXCL501 into in-care and at-home settings**

If approved, launch with established payor contracts, experienced (hospital) advocates, and DTC/PR campaign

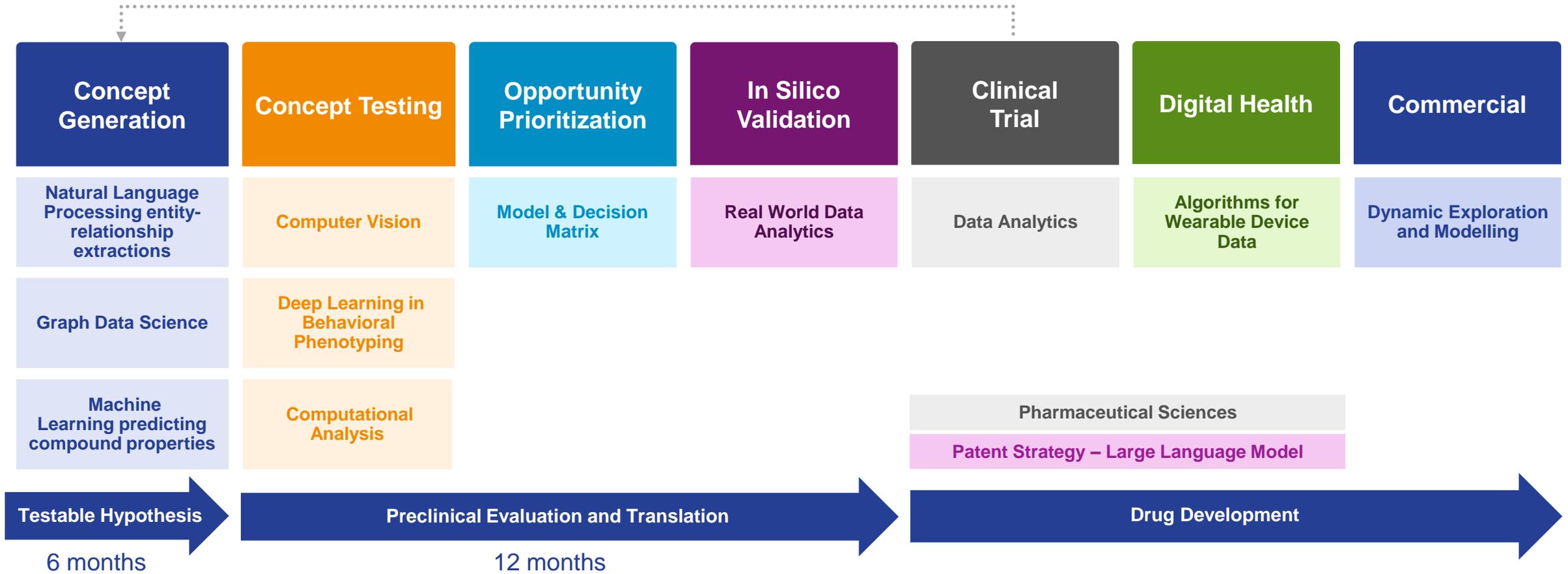
# AI-Driven Drug Re-innovation Platform

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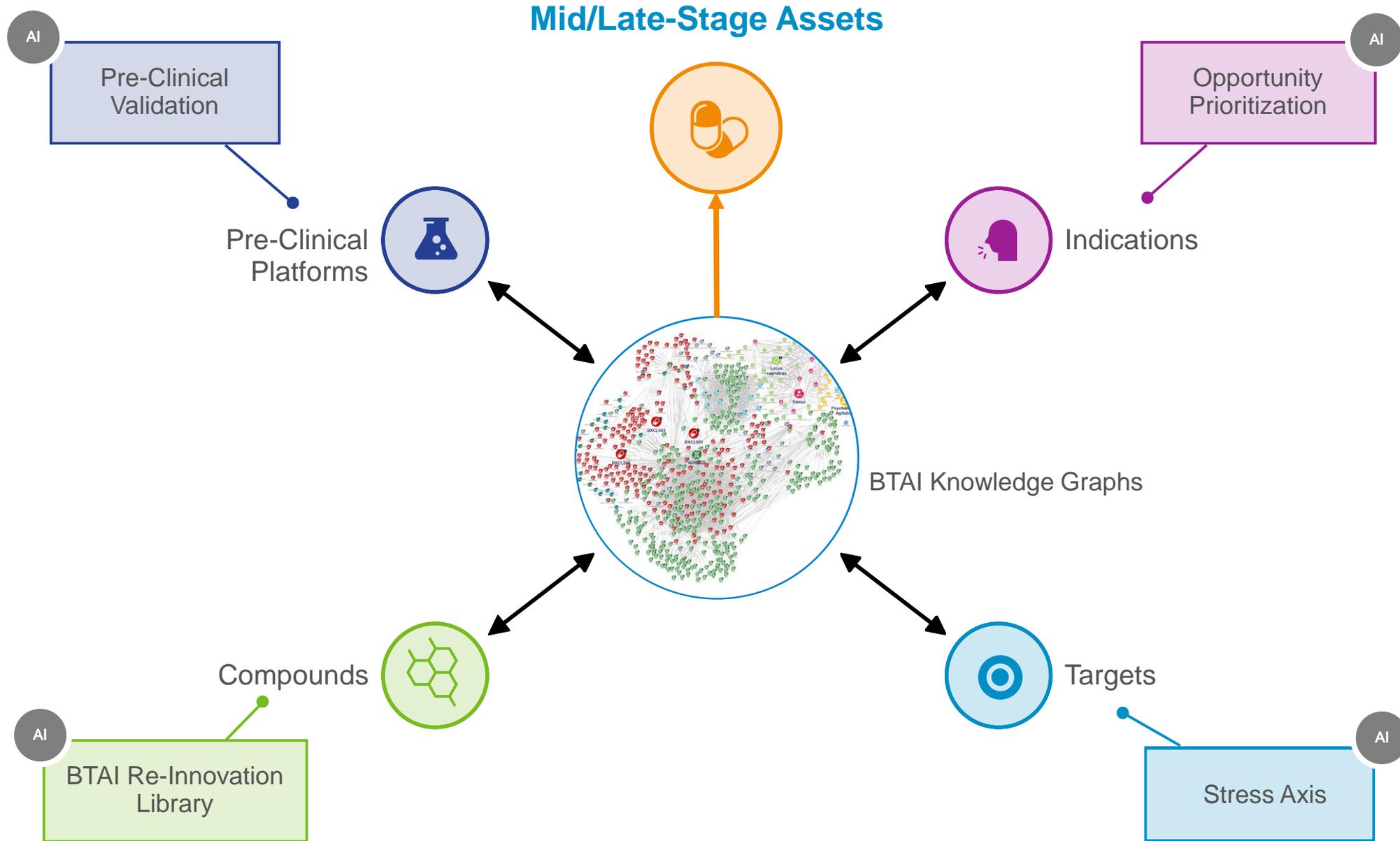
# AI Strategy to Accelerate Drug Re-Innovation Process

From product concept to first-in-human clinical trials using composite AI

BioXcel Therapeutics AI and Analytics

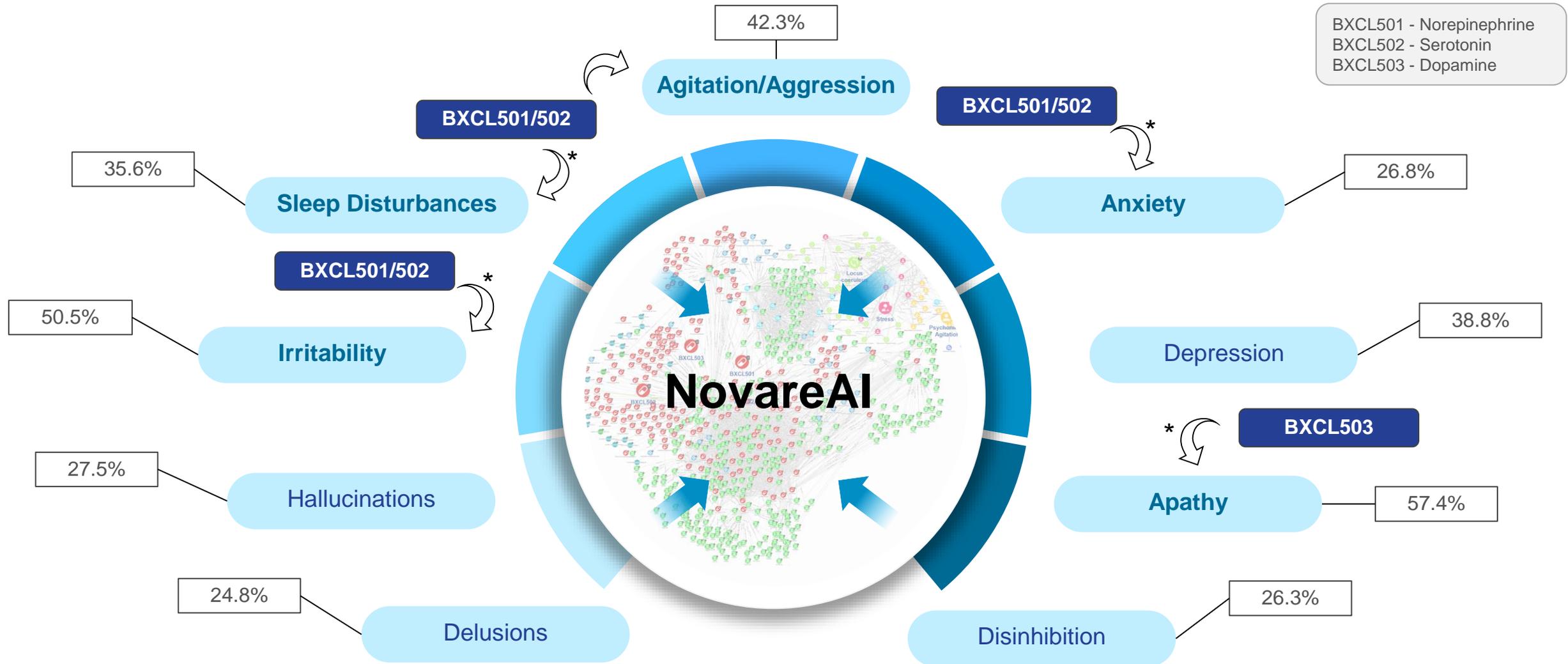


# NovareAI: Ecosystem for Drug Discovery and Development



# Behavioral and Psychological Symptoms in Alzheimer's Disease

## Identifying targets and compounds designed to address unmet medical needs in dementia



\* Denotes a potential role for BXCL502 and BXCL503 in addressing these symptoms  
 The safety and efficacy of investigational agents and/or investigational uses of approved products have not been established.  
 Prevalences derived from Laganà et al., Neuropsychiatric or Behavioral and Psychological Symptoms of Dementia (BPSD): Focus on Prevalence and Natural History in Alzheimer's Disease and Frontotemporal Dementia; Front Neurol 2022;13 832199

# **BXCL502: A Novel Agent for Treatment of Chronic Agitation in Dementia**

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# BXCL502 Presents a Compelling Value Proposition

Formulation studies are ongoing



**New Chemical Entity**

**BXCL502 is a novel formulation of latrepirdine and a metabolic stabilizer**



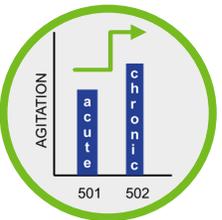
**Improved Understanding of Proposed Mechanism**

**Potentially blocks excessive signaling mediated by 2 neurotransmitters: serotonin and norepinephrine (noradrenaline)**



**Re-Innovation of Latrepirdine**

**Improved PK results suggest potential for once-daily dosing, which could be suitable for chronic agitation**

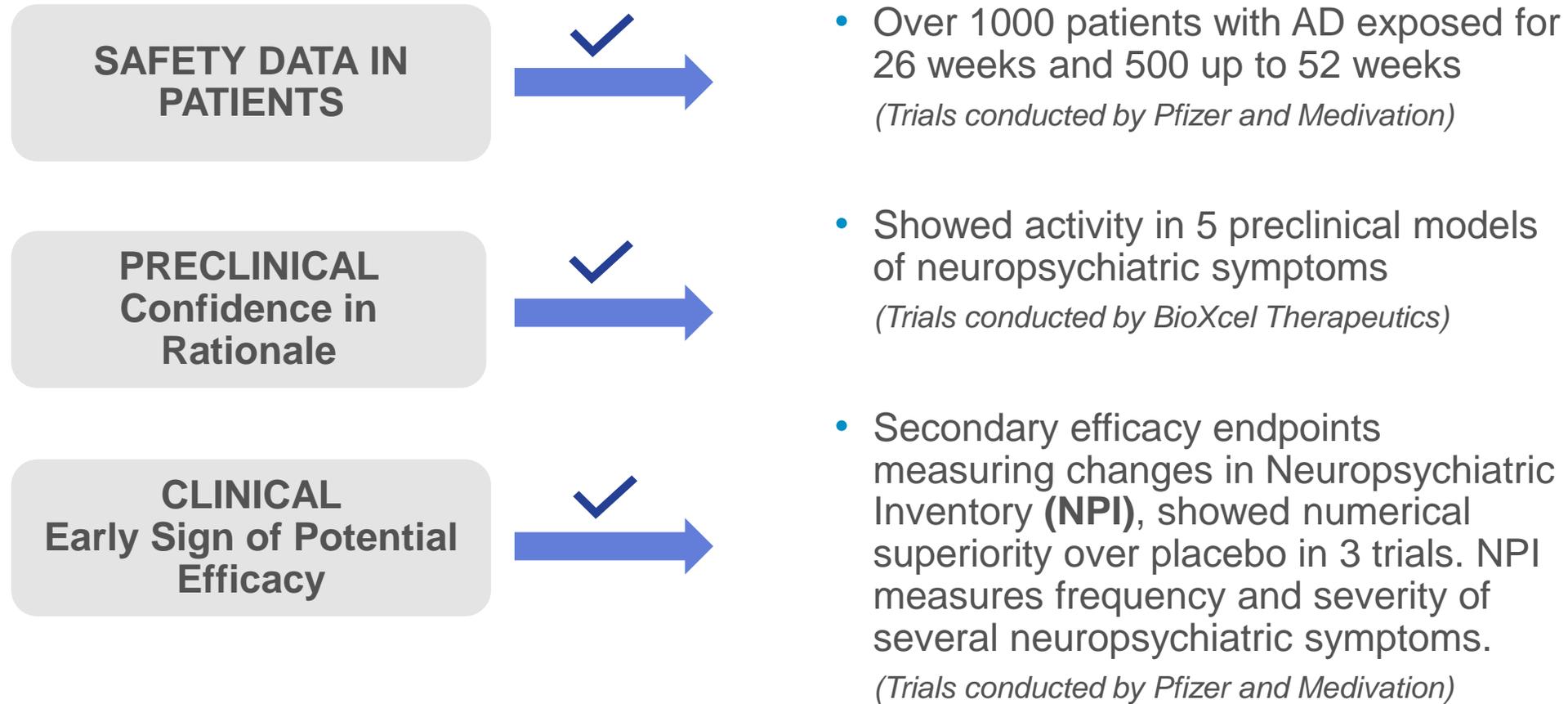


**Synergistic with Portfolio**

**Expanding agitation development programs from episodic to chronic**

# Latrepirdine (Dimebon): Clinical Safety Data, Preclinical Confidence in Rationale, and Early Sign of Potential Efficacy

Data support development for treatment of neuropsychiatric symptoms associated with dementia



# Recent Examples of Successful CNS Drug Re-Innovation

DRUG/COMPANY	CHALLENGE	SOLUTION	STATUS
<b>Dextromethorphan</b> Axsome Therapeutics	Low bioavailability & metabolites that may cause side effects	Bupropion significantly increases dextromethorphan bioavailability	Successful clinical trial/depression
<b>Xanomeline</b> Karuna Therapeutics	Peripheral side effects	Block peripheral effects with trospium	Successful clinical trial/schizophrenia
<b>Dexmedetomidine</b> IGALMI™ BioXcel Therapeutics	Poor oral bioavailability (<20%)	Use sublingual film to increase bioavailability (sublingual bioavailability ~70%)	Approved to treat adults with agitation associated with schizophrenia or bipolar I or II disorder

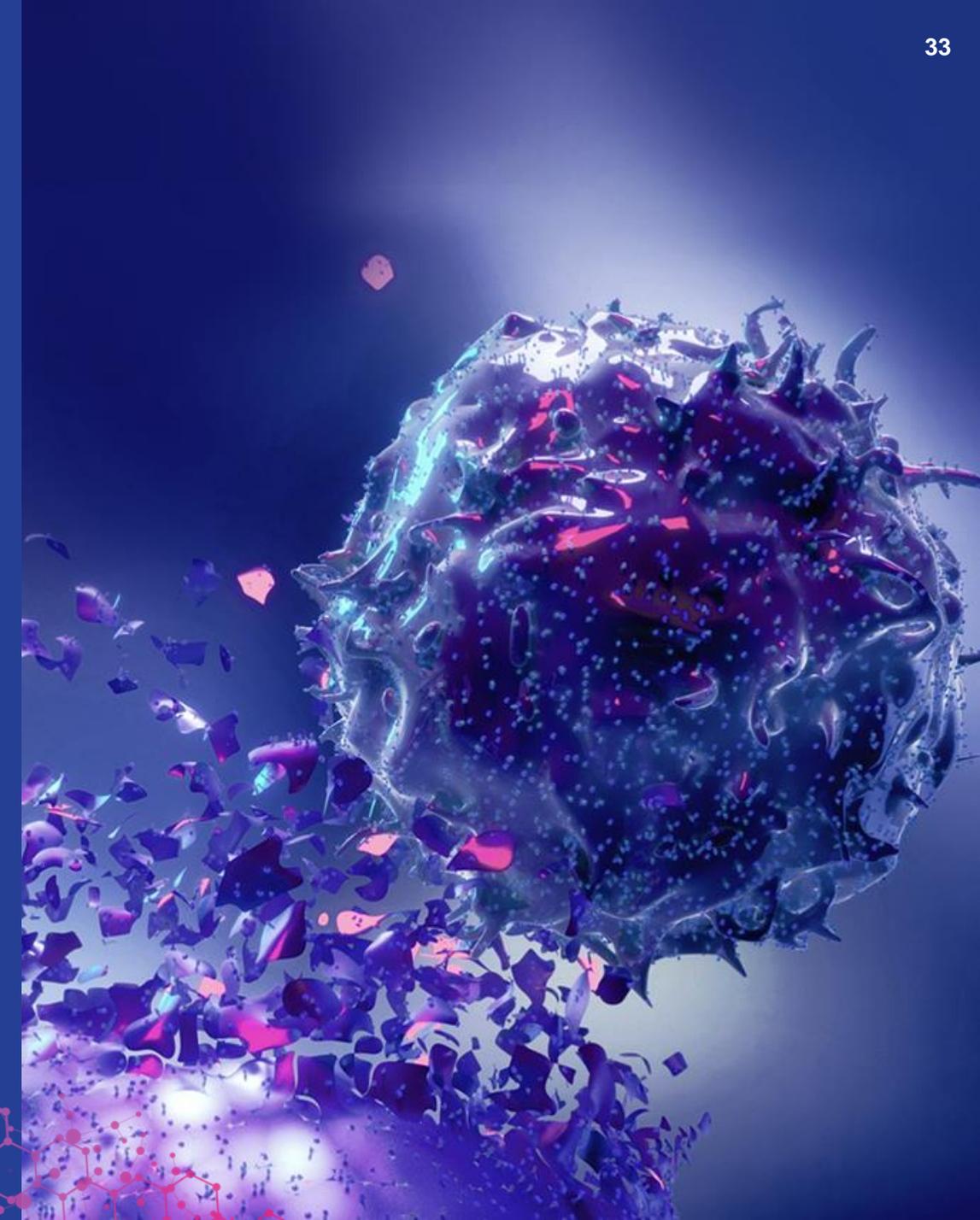
***Latrepirdine + “Metabolic Stabilizer” = BXCL502***

# Immuno-Oncology

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# OnkosXcel Therapeutics™

A subsidiary of BioXcel Therapeutics, Inc.



# BXCL701: Strong Value Proposition in Hard-to-Treat Tumors

## Novel Mechanism of Action Data Published in JITC

- One of the most clinically advanced oral innate immune activators<sup>1</sup>
- Designed to activate inflammasome via DPP8/9 inhibition

## Clinical Proof of Concept Cold Tumors

- Compelling survival benefit and antitumor activity in two cold tumor types: mCRPC SCNC and adenocarcinoma
- FDA fast track designation for BXCL701 for treatment of SCNC<sup>2</sup>
- 800+-subject clinical safety database

## Leadership Position in Innate Immunity DPP8/9 Biology

*FortySeven*  
acquired for ~\$5B  
by GILEAD

*Trillium*  
acquired for ~\$2.3B  
by Pfizer

Scarcity of assets  
in innate immunity

## Exploring Strategic Options

- Could include, among other options, potential financing, strategic partnership, or M&A

BXCL701 (talabostat) is an investigational agent. The safety and efficacy have not been established.

mCRPC = metastatic castration-resistant prostate cancer | SCNC = small cell neuroendocrine prostate cancer

1. National Library of Medicine. Accessed January 4, 2024, [clinicaltrials.gov](https://clinicaltrials.gov).

2. [Company announcement](#), February 12, 2024: FDA designated as a Fast Track development program the investigation of BXCL701 in combination with a CPI for the treatment of patients with metastatic small cell neuroendocrine prostate cancer (SCNC) with progression on chemotherapy and no evidence of microsatellite instability.

# Immuno-Oncology Clinical Development

Compound	Proposed Indication	Preclinical	Phase 1	Phase 2	Phase 3	Expected Upcoming Milestone	Collaborator
<b>BXCL701</b> Company-sponsored trials	Small Cell Neuroendocrine Prostate Cancer (SCNC)					FDA Meeting	13 centers US / UK
	Small Cell Lung Cancer (SCLC)					Initiate Phase 1b/2	
<b>BXCL701</b> Investigator-sponsored trials	Metastatic Pancreatic Ductal Adenocarcinoma					Phase 2 readout	Georgetown Lombardi Comprehensive Cancer Center Supply agreement: Merck & Co.
	Acute Myeloid Leukemia (AML)					Phase 1b readout	Dana-Farber Cancer Institute
<b>BXCL702</b> <b>BXCL701</b> follow-on/ novel DPP inhibitor	Solid Tumors					Candidate nomination	

As of April 22, 2024

The safety and efficacy of these investigational agents have not been established.

# Thank you!

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