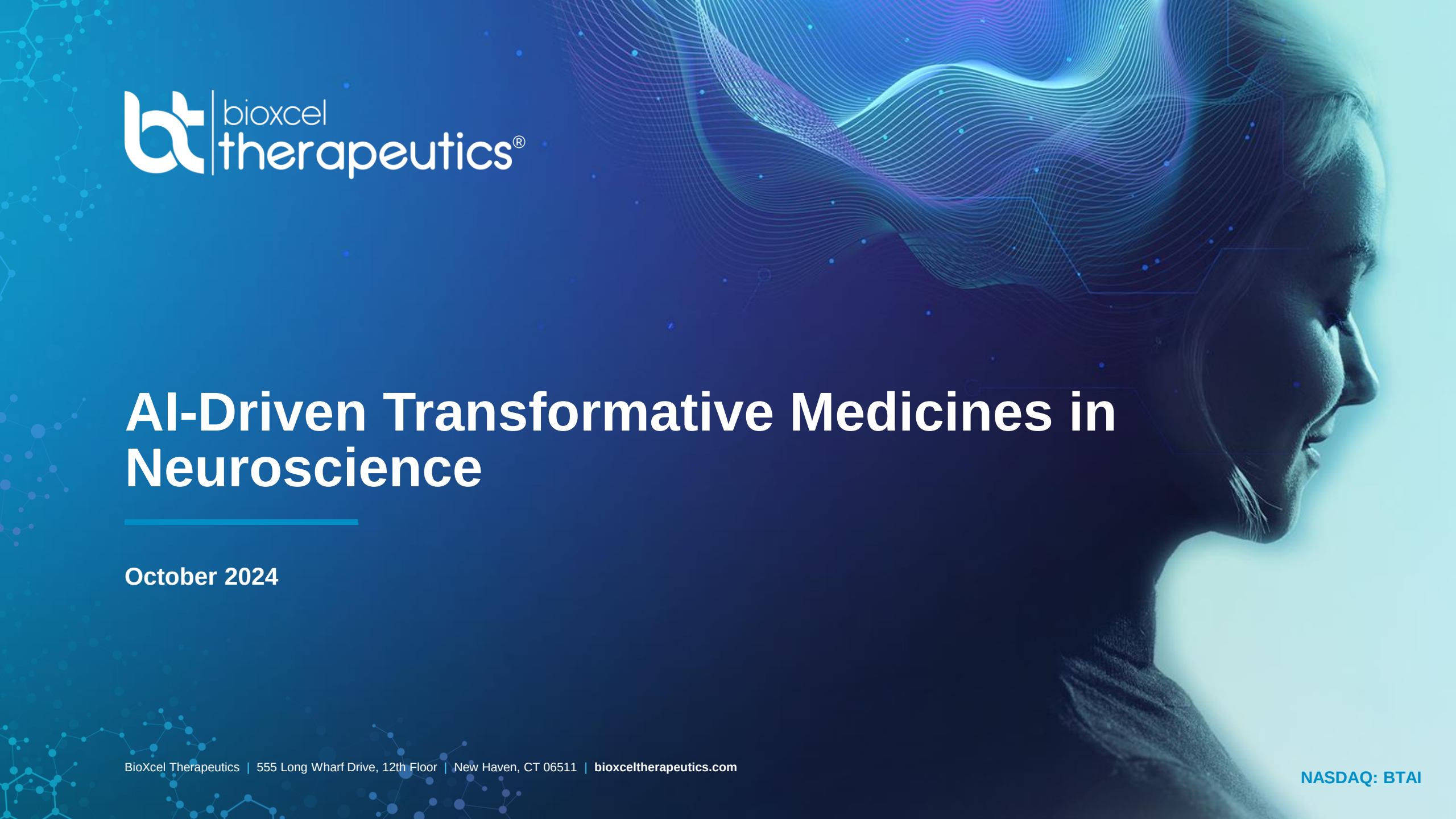

AI-Driven Transformative Medicines in Neuroscience

October 2024

BioXcel Therapeutics | 555 Long Wharf Drive, 12th Floor | New Haven, CT 06511 | **bioxceltherapeutics.com**

NASDAQ: BTAI

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; the initiation of any timing of any topline date from the SERENITY program; 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 the Company’s AI platform; 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, as such factors may be updated from time to time in its other filings with the SEC, including without limitation its Quarterly Report on Form 10-Q for the quarterly period ended June 30, 2024, which are accessible on the SEC’s website at www.sec.gov and the Investors section of the Company’s 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 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.

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Corporate Overview

About BioXcel Therapeutics

Founded: 2017

IPO: 2018

Ticker: BTAI (Nasdaq)

Headquarters: New Haven, CT

Our Mission

Develop transformative medicines in neuroscience utilizing artificial intelligence

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Advancing our Land and Expand Strategy with BXCL501

Land

Agitation Associated With Bipolar Disorders or Schizophrenia

Medical Supervision

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

~16M

BPD/SCZ Institutional Episodes[1-3]

Expand*

Agitation Associated with Bipolar Disorders or Schizophrenia

(at-home setting)

Agitation Associated with Alzheimer's Dementia

BXCL501 Phase 3 SERENITY and TRANQUILITY Programs

Up to ~23M*

BPD/SCZ Agitation Episodes[1-3]

Up to ~100M*

Alzheimer's* Agitation Episodes[1]

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.

* The safety and efficacy of investigational products and/or investigational uses of approved products have not been established. Actual addressable market may be smaller

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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™ 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.

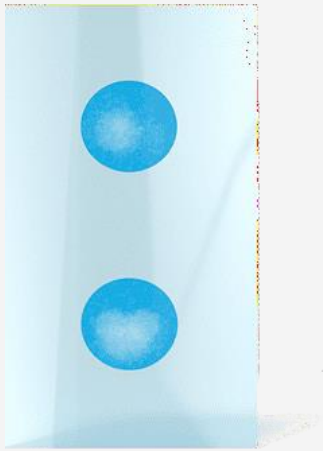
These are not all the possible side effects of IGALMI. Patients should speak with their healthcare provider for medical advice about side effects.

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 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.

IGALMI™ is a Noninvasive, Self-Administered Film[1]



- **Patient administered** under the supervision of a healthcare provider who should monitor vital signs and alertness to prevent falls and syncope[1]
- **Absorption** of dexmedetomidine into the bloodstream and quantifiable in plasma after 5 to 20 minutes with a T-max of 2 hours and half life of 2.8 hours[1]
- IGALMI **significantly reduced agitation** as early as 20-30 minutes following administration[1*]
- Mucoadhesive, so it **cannot be spit out or swallowed**[1-3]
- **Sublingual** or **buccal** placement[1]
- IGALMI is **non-scheduled**[1]

TWO DOSAGE STRENGTHS

SELF-ADMINISTRATION

DOSAGE RECOMMENDATIONS

Please see Important Safety Information at end of presentation and full Prescribing Information at www.igalmi.com

References: 1. Miller CWT, et al. *West J Emerg Med*. 2020;21(4):841-848. doi:10.5811/westjem.2020.4.45779 **2.** IGALMI. Package insert. BioXcel Therapeutics, Inc; 2022. **3.** Data on file. BXCL501-301 CSR (SERENITY I). BioXcel Therapeutics, Inc; January 2021.

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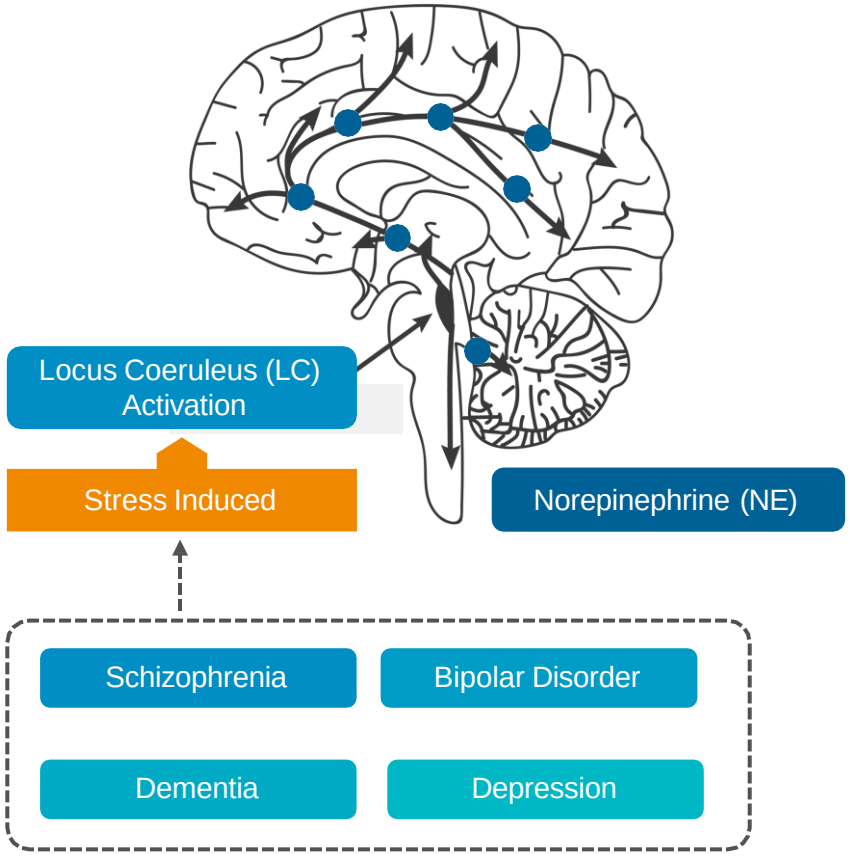
Agitation Mechanism and BXCL501 (Dexmedetomidine) MOA

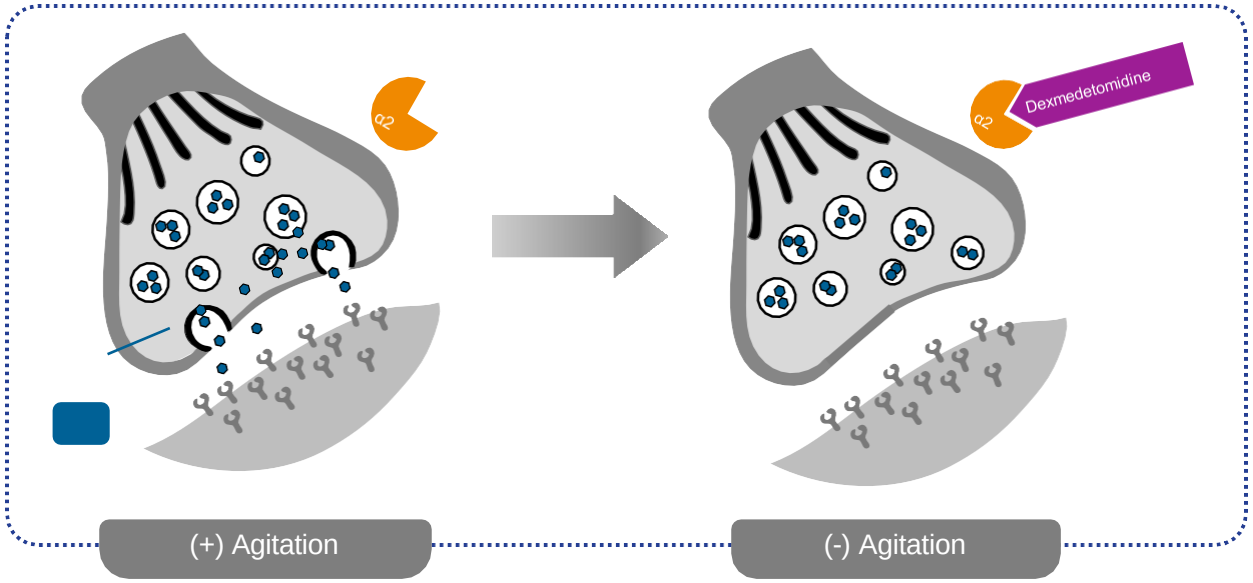
Disorders like schizophrenia, bipolar, and dementia can affect the hyperarousal pathway, causing LC neurons to fire in a tonic mode causing fight-or-flight response

Hyper-Arousal Physiology



Dexmedetomidine is one of the most potent, selective, full agonists at Alpha-2A receptors
Dexmedetomidine has been shown to reduce hyper-arousal through selective agonist action at presynaptic Alpha-2A Adrenergic Autoreceptors.

Dexmedetomidine MoA



Neuroscience Development Portfolio

Compound	Indication/Proposed Indication	Preclinical	Phase 1	Phase 2	Phase 3	Registration	Marketed
IGALMI™ (dexmedetomidine) sublingual film	Acute treatment of agitation associated with schizophrenia or bipolar I or II disorder in adults under healthcare provider supervision						
BXCL501 Company-sponsored trials*	SERENITY PROGRAM Acute treatment of agitation associated with bipolar disorders or schizophrenia (at home)						
	TRANQUILITY PROGRAM Acute treatment of agitation associated with Alzheimer's dementia						
BXCL501 Investigator-sponsored trials*	Opioid use disorder[1]						
	Alcohol use disorder with comorbid post-traumatic stress disorder[2]						
	Acute stress disorder[3]						
BXCL501	Anxious depression**						
BXCL502 (latrepirdine)	Chronic agitation in dementia**						
Candidate BXCL503	Apathy in dementia**						
Candidate BXCL504	Aggression in dementia**						

1 Collaborator: Columbia University 2 Collaborator: Yale University Medical School 3 Collaborator: University of North Carolina at Chapel Hill

*The safety and efficacy of investigational agents and/or investigational uses of approved products have not been established **Development paused due to Strategic Reprioritization announced on Aug. 14, 2023

As of October 15, 2024

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Leadership Expertise

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

BioXcel Therapeutics
inpharmatica
JUBILANT LIFESCIENCES
CuraGen Corporation

Richard I. Steinhart
Senior Vice President
& Chief Financial Officer

MELA Sciences
remedy PHARMACEUTICALS
pwc

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

Bristol Myers Squibb
AstraZeneca
BioXcel Therapeutics

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

sanofi
Genentech
A Member of the Roche Group
gsk

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

reckitt
BAYER
Berlex Pharmaceuticals

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

Ortho Mcneil Janssen Pharmaceuticals
Bristol Myers Squibb
Alkermes

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

Otsuka
janssen
Bristol Myers Squibb

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Strong Value Proposition and Long-Term Growth Potential

Well-positioned to advance clinical programs and accelerate AI-based drug re-innovation

Initiated SERENITY At-Home pivotal Phase 3 safety trial

Acute treatment of agitation associated with bipolar disorders or schizophrenia (at home)

Submitted protocol to FDA for TRANQUILITY In-Care pivotal Phase 3 trial

Acute treatment of agitation associated with Alzheimer's dementia

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

Maintaining market presence

Unique AI capability to build innovative neuroscience pipeline

Includes BXCL502 (chronic agitation) and BXCL503 (apathy in dementia)

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

SERENITY Program

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Agitation: Relatively Common and Difficult-to-Manage[1]

Debilitating for Patients and Threatening for Healthcare Providers

Characterized by recurring episodes[1]

Symptoms differ by patient, vary between episodes, and range from mild to severe[2-7]

Multi-billion-dollar healthcare burden[8]

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[9]

Current treatment approaches:

- May involve physically restraining patients[10]
- Over-sedating therapies such as antipsychotics and benzodiazepines[10]

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..Dundar 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

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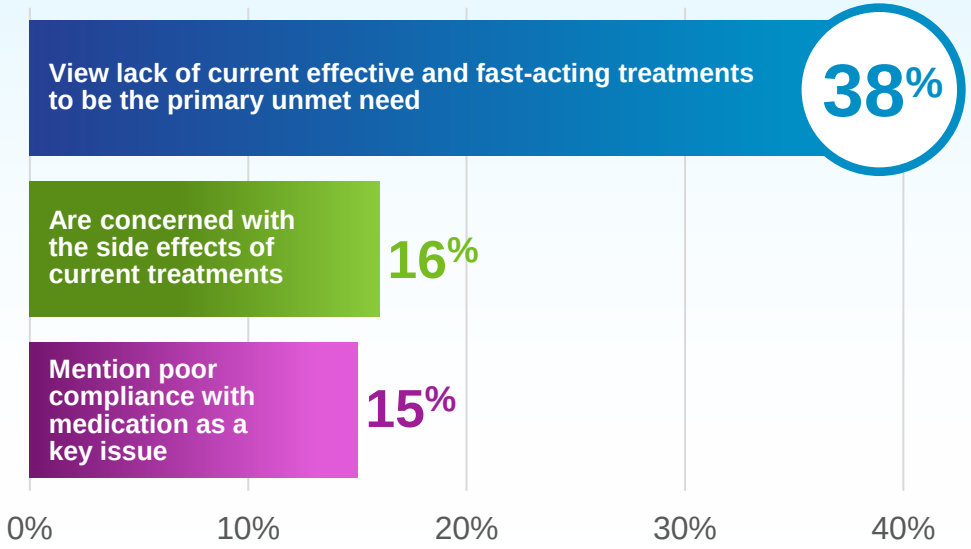
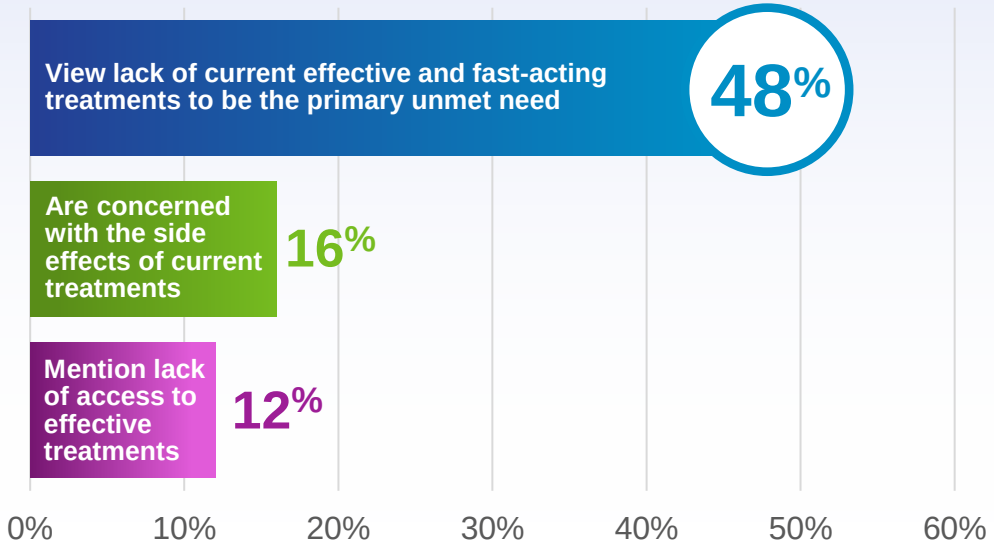
Physician Market Research Cites Need for Effective, Fast-acting, More Tolerable Treatment Options

Key unmet needs in bipolar disorders



"The available drugs either work too slowly or have unacceptable side effects."
– Psychiatrist

Key unmet needs in schizophrenia



"Other than the sedative effects of antipsychotics, there really aren't any good options for managing agitation without just making the patient drowsy."
– Psychiatrist

Source: HCP Demand Study Oct 2022 Quantitative research n=90

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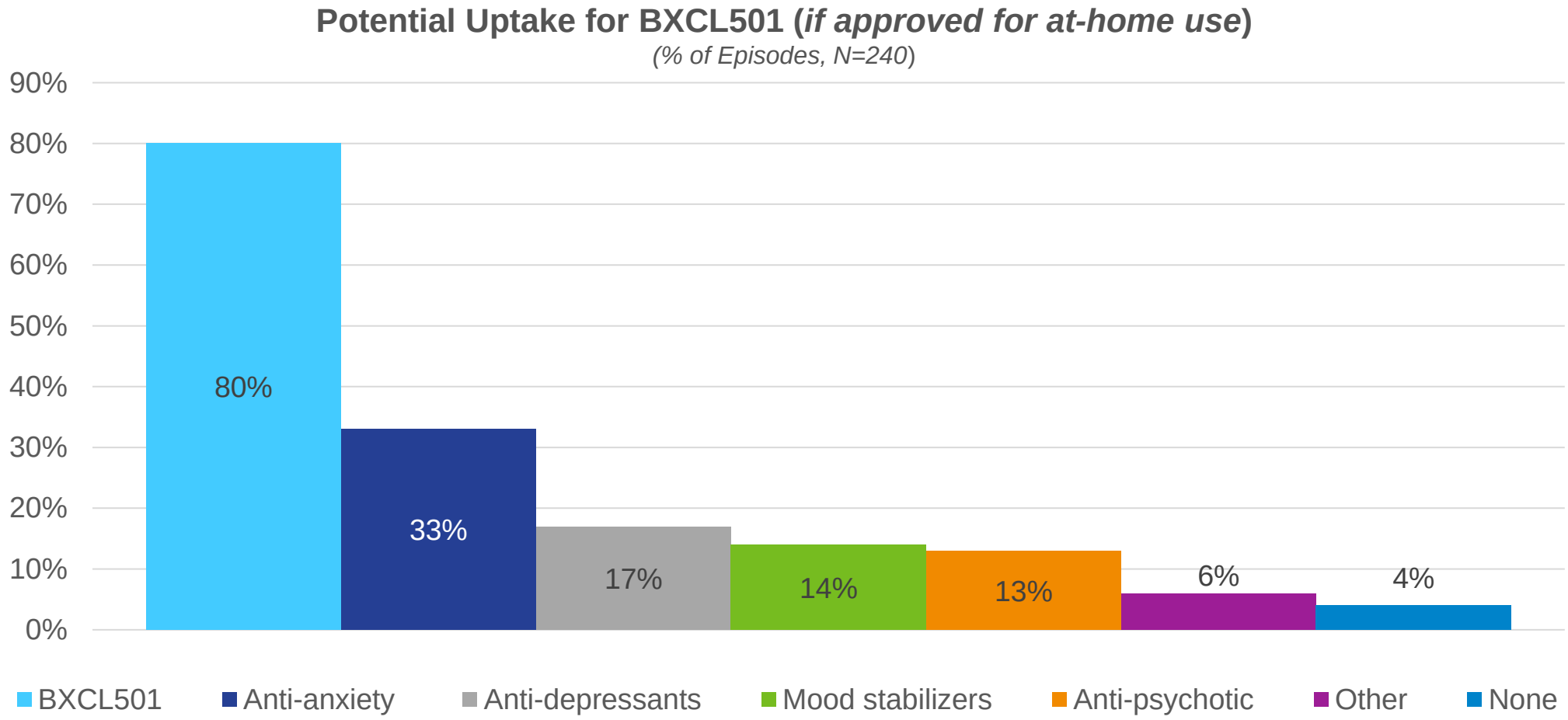
Potential Patient Use of BXCL501 At Home

When shown product profile stimulus, patients stated they would use a product with the BXCL501 target profile for 80% of their bipolar/schizophrenia agitation episodes



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 response below for reference.

Source: InVibe Feb 2023

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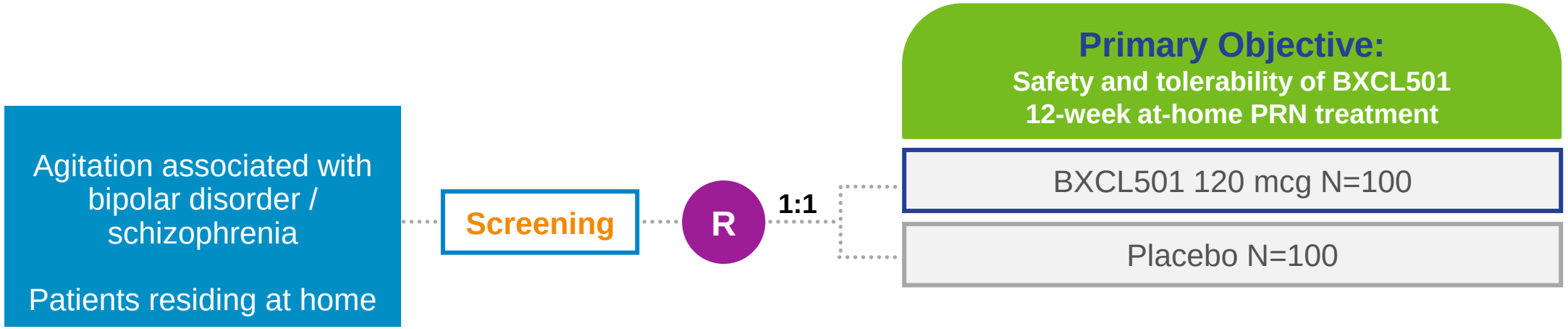
SERENITY Program Seeks Potential IGALMI™ Label Expansion

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

SERENITY At-Home Trial Design



- **Design:** double-blind, placebo-controlled trial to evaluate the safety of a 120 mcg dose of BXCL501 in the home setting
- **Recruitment Criteria**
  - Patients with or without co-resident family members/informants
  - 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
- **Primary Objective:** Safety
- **Exploratory Endpoint**: Patients or caregivers/informants will complete a modified clinical global impression of change (mCGI-C) two hours after dosing to evaluate their impression of use in outpatient setting

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Positive Topline Results from Post-Marketing Requirement Study Evaluating PRN Treatment of IGALMI™

Study achieved its objective and demonstrated no evidence of tachyphylaxis, tolerance, or withdrawal with 180 mcg dose (highest approved dose)

Efficacy Measurements*

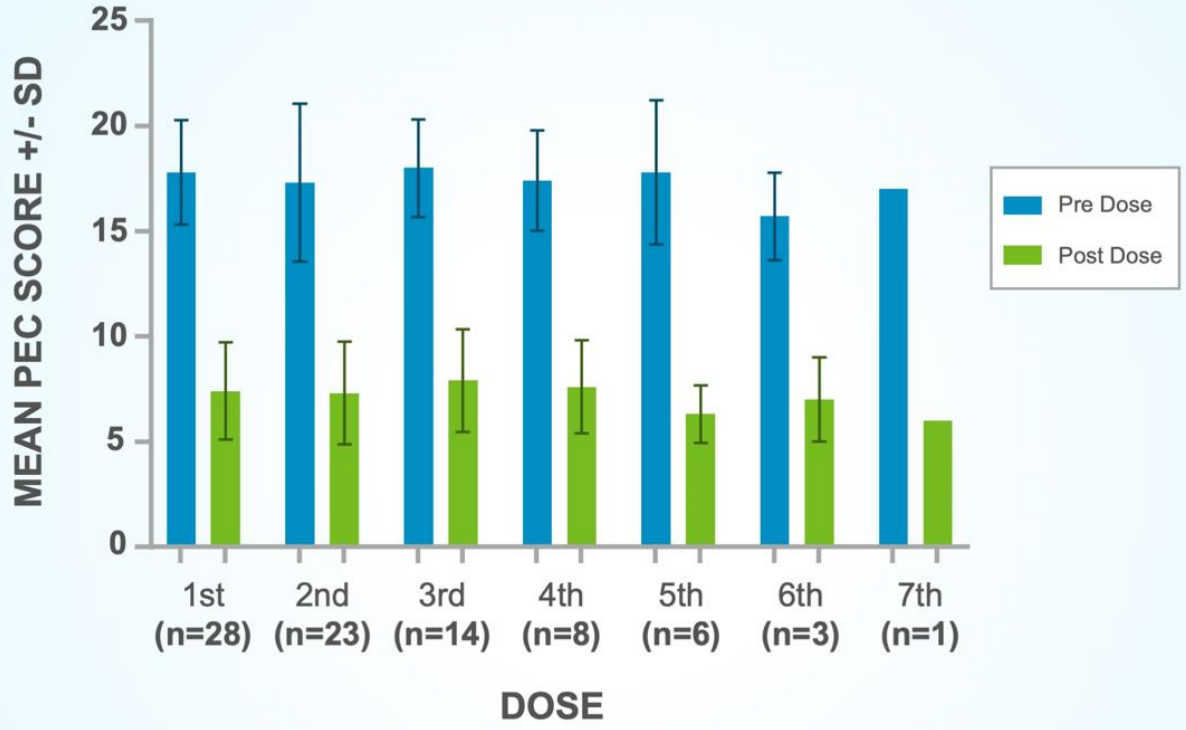
- Mean PEC score reduction was observed following all doses of IGALMI administered as needed over the treatment period).
- All patients showed improvement in agitation symptoms as assessed by the CGI-I Scale for all doses administered as needed over the treatment period.
- Prior to treatment with IGALMI, most patients exhibited mild to moderate agitation as assessed by the Agitation Calmness Evaluation Scale (ACES). ACES scores post-dose revealed a marked calming effect with no patient experiencing unarousable somnolence (ACES score 9).
- In addition, no withdrawal or rebound phenomena were observed.

Safety and Tolerability Results

- The 180 mcg dose of IGALMI was generally well tolerated and showed favorable safety results in treating patients with frequent episodes of agitation.
  - There were no discontinuations due to adverse events.
  - No serious adverse events were reported.

*Although PMR study was not statistically powered to evaluate repeat-dose efficacy, changes in agitation were assessed through the Positive and Negative Syndrome Scale-Excitatory Component (PEC or PANSS-EC) Score and the Clinical Global Impressions – Improvement (CGI-I) Scale, the same measures as used in previous Phase 3 studies.

Mean PEC Score Changes During PMR Study Period



Subjects were dosed for up to 7 days with IGALMI. All subjects (n=28) received a first dose. The number of subjects that received a second, third or more doses is indicated below the dose number. Graph shows the mean PEC score for each dose, with standard deviation, for each dose (first, second third, etc.). PEC was assessed predose and 2 hours post dose.

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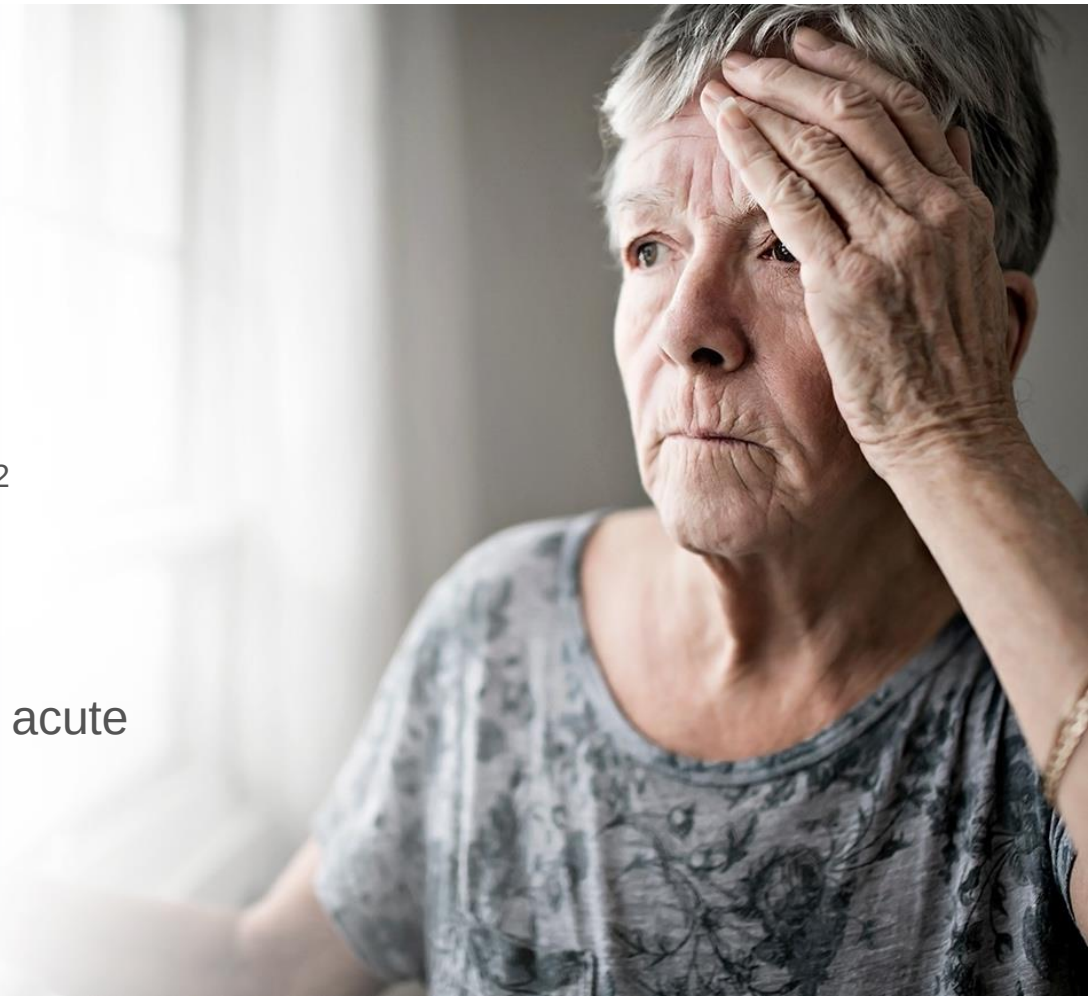
Acute Treatment of Agitation Associated with Alzheimer's Dementia (AAD)

TRANQUILITY Program

AAD is Debilitating for Patients and a Burden for Caregivers

Agitation cited as a top driver in decision to move a patient from home setting to residential care facility[1]

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



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 Geriatri Psychiatry. 2019; 34: 420-431.
3. Joint Meeting of the Psychopharmacologic and the Peripheral and Central Nervous System Drugs Advisory Committee Meeting April 14th, 2023

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TRANQUILITY Program Offers Potential Opportunity for Treating AAD

- Details of requirement for long-term safety data will be discussed with the FDA in the future**

* 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

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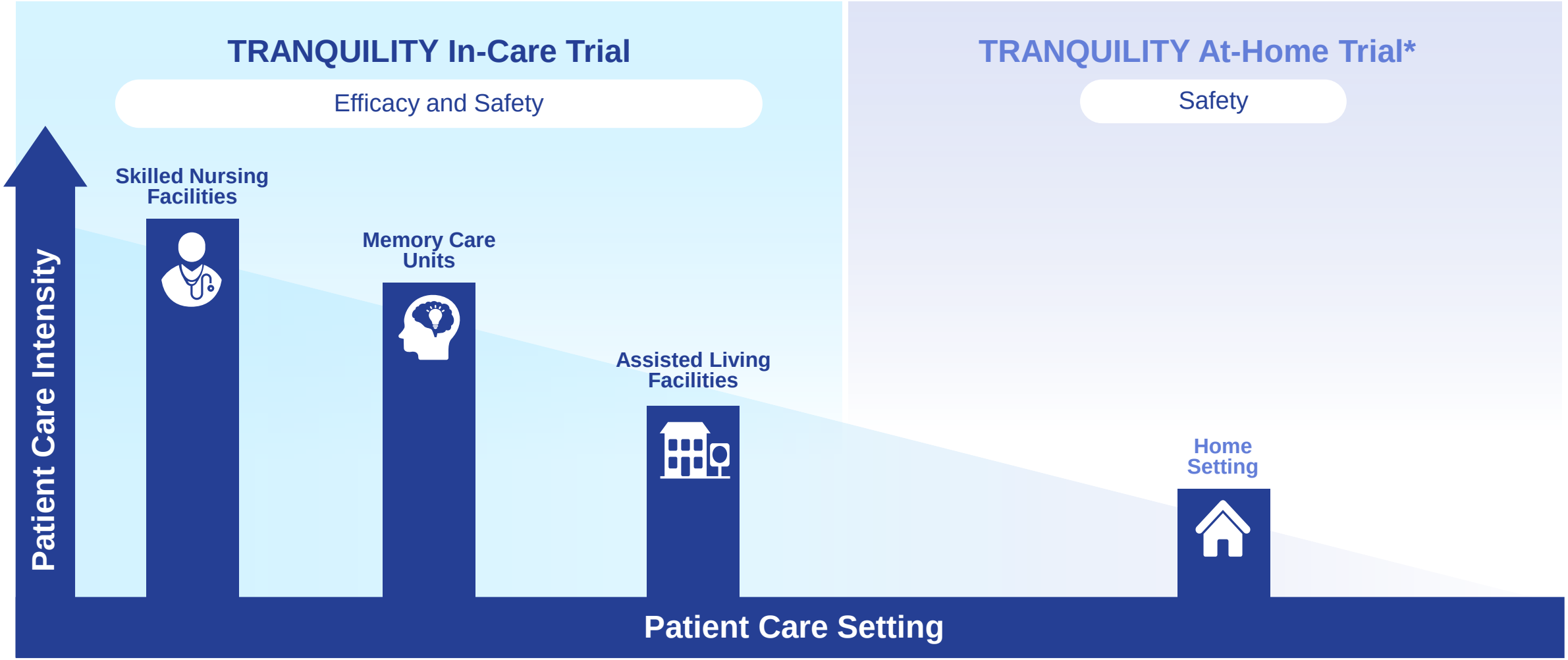
Evaluating BXCL501 for AAD in High to Low Care Settings

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)

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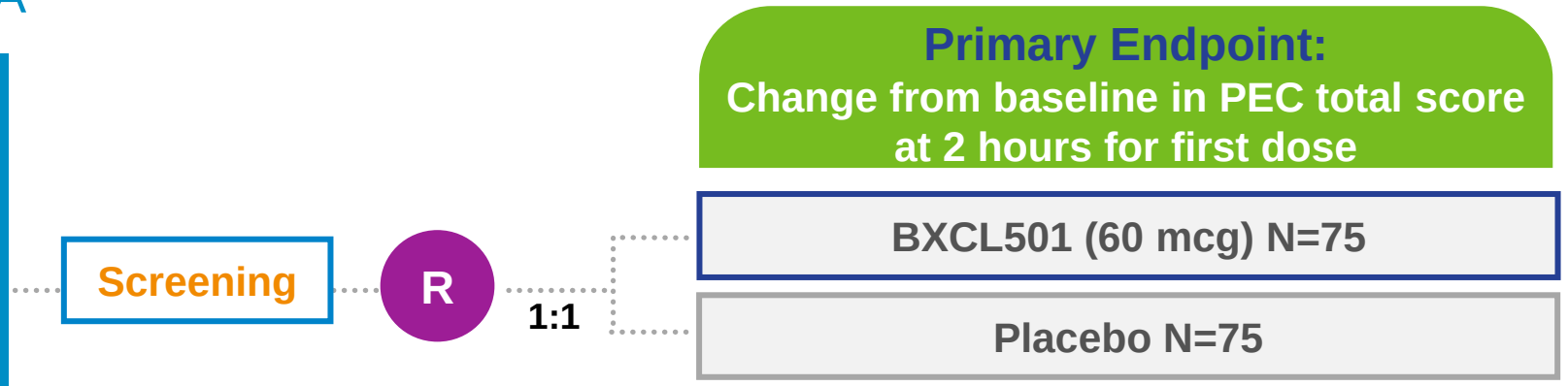
TRANQUILITY In-Care Trial Design*

Protocol submitted to FDA

Agitated patients with dementia and probable Alzheimer's disease

Patients residing in care facilities



- **Design:** Randomized, double-blind, placebo-controlled, parallel group trial
- **Power:** Over 80% power
- **Inclusion Criteria**
  - Patients with probable AD (mild, moderate, or severe; MMSE ≤ 25), who experience agitation, and reside in skilled nursing facilities, memory care units, or assisted living facilities
  - PEC total score ≥14 prior to randomization
- **Primary Endpoint:** Change from baseline in PEC total score at 2 hours for first dose
- **Study Duration:** 12 weeks with assessment of continued efficacy (episodes assessed pre-post by qualified PEC rater)

*For illustrative purposes only: The FDA has not provided feedback on the protocol.

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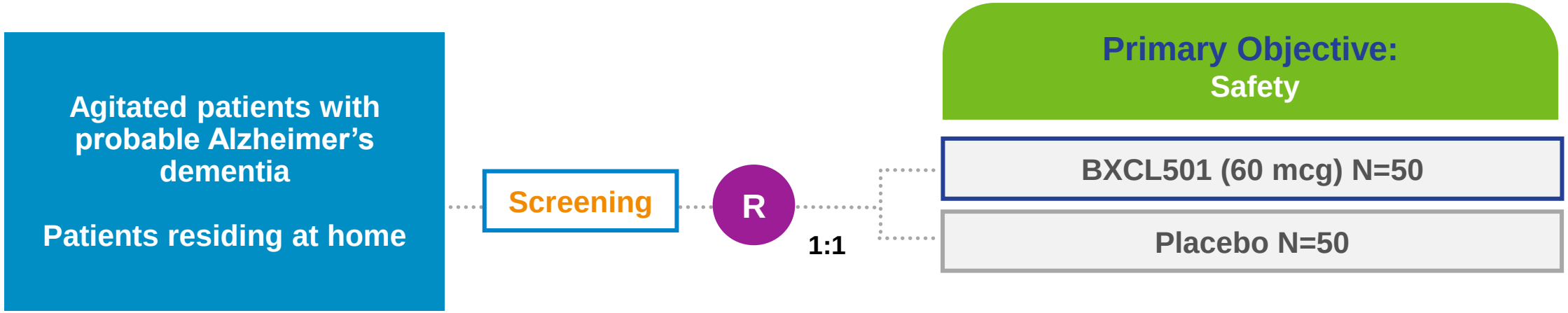
Preliminary TRANQUILITY At-Home Trial Design*



- **Design:** Randomized, double-blind, placebo-controlled, parallel group trial
- **Primary Objective:** Safety and tolerability of BXCL501 60 mcg
- **Inclusion Criteria**
  - Patients with probable AD (mild, moderate, or severe; MMSE ≤ 25), who experience agitation and reside at home
  - 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 pending further discussions with the FDA.

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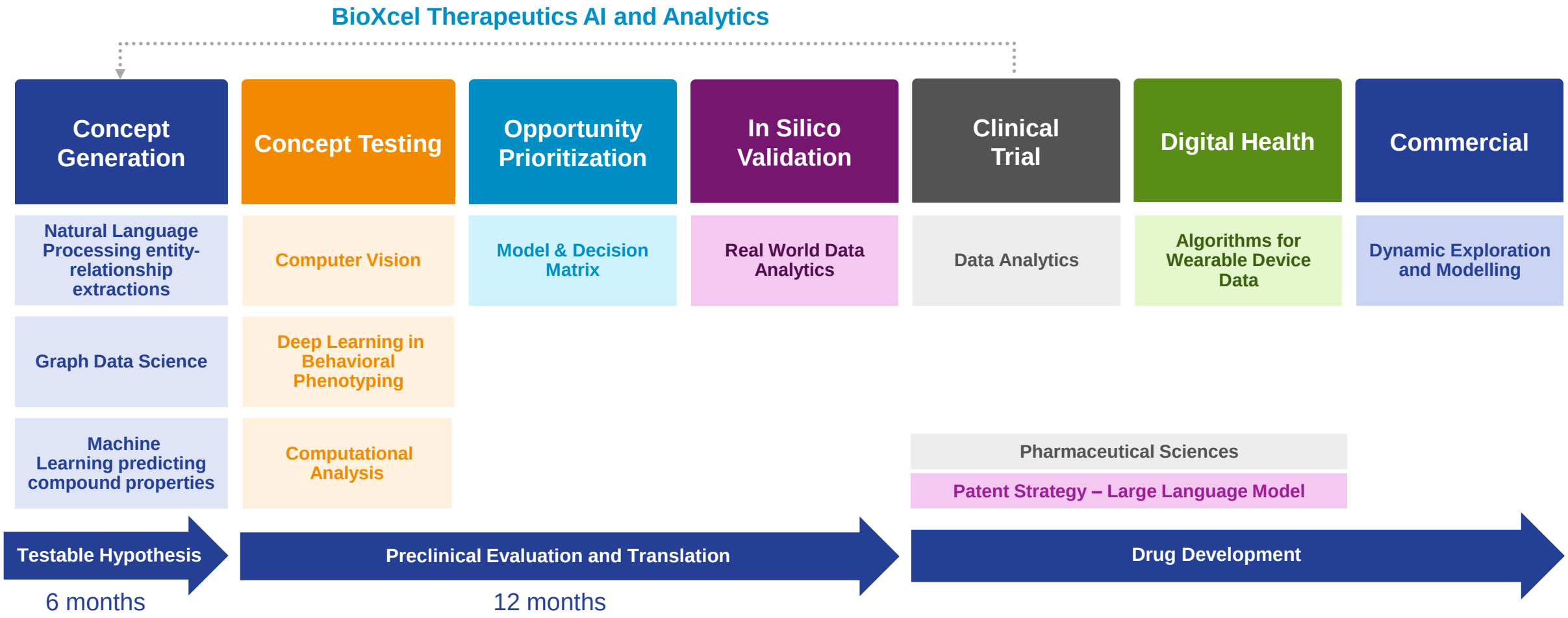
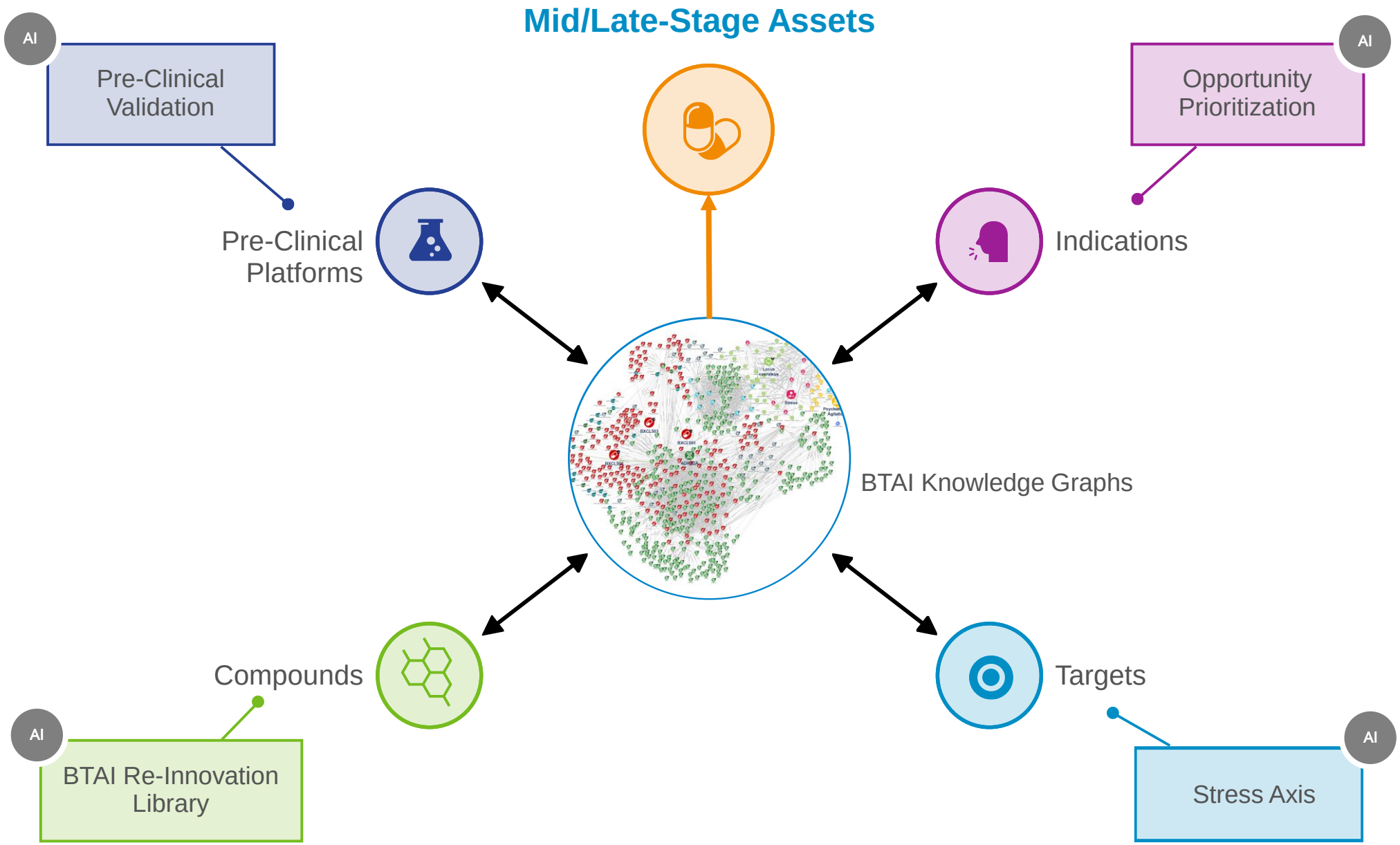
AI-Driven Drug Re-innovation Platform

AI Strategy to Accelerate Drug Re-Innovation Process

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

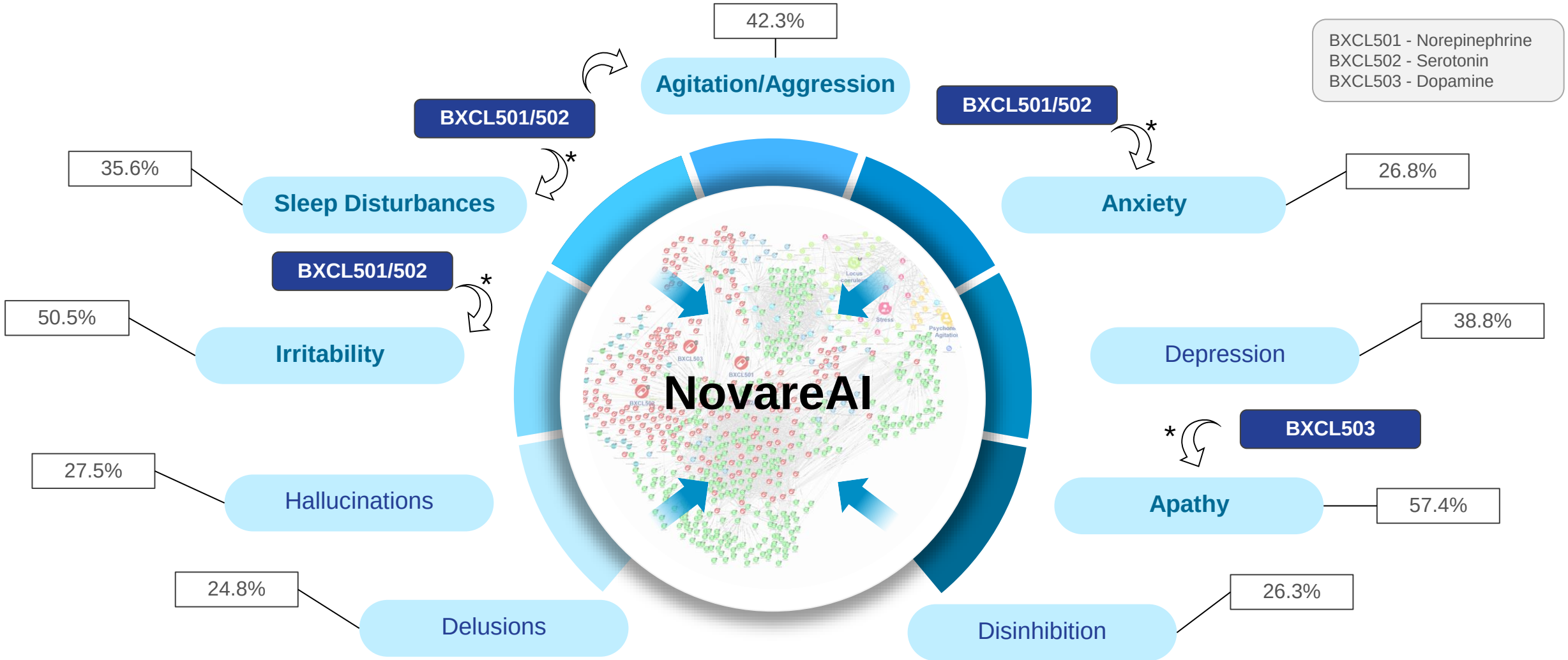


NovareAI: Ecosystem for Drug Discovery and Development



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

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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

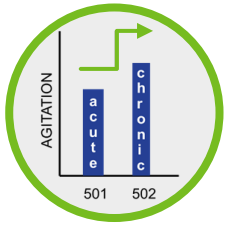
BXCL502 is a novel formulation of latrepirdine and a metabolic stabilizer

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

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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

SAFETY DATA IN PATIENTS

- Over 1000 patients with AD exposed for 26 weeks and 500 up to 52 weeks

  (Trials conducted by Pfizer and Medivation)

PRECLINICAL
Confidence in Rationale

- Showed activity in 5 preclinical models of neuropsychiatric symptoms

  (Trials conducted by BioXcel Therapeutics)

CLINICAL
Early Sign of Potential Efficacy

- Secondary efficacy endpoints measuring changes in Neuropsychiatric Inventory **(NPI)**, showed numerical superiority over placebo in 3 trials. NPI measures frequency and severity of several neuropsychiatric symptoms.

  (Trials conducted by Pfizer and Medivation)

Recent Examples of Successful CNS Drug Re-Innovation

DRUG/COMPANY	CHALLENGE	SOLUTION	STATUS
Dextromethorphan axsome	Low bioavailability & metabolites that may cause side effects	Bupropion significantly increases dextromethorphan bioavailability	Successful clinical trial/depression
Xanomeline KARUNA THERAPEUTICS	Peripheral side effects	Block peripheral effects with trospium	Successful clinical trial/schizophrenia
Dexmedetomidine 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

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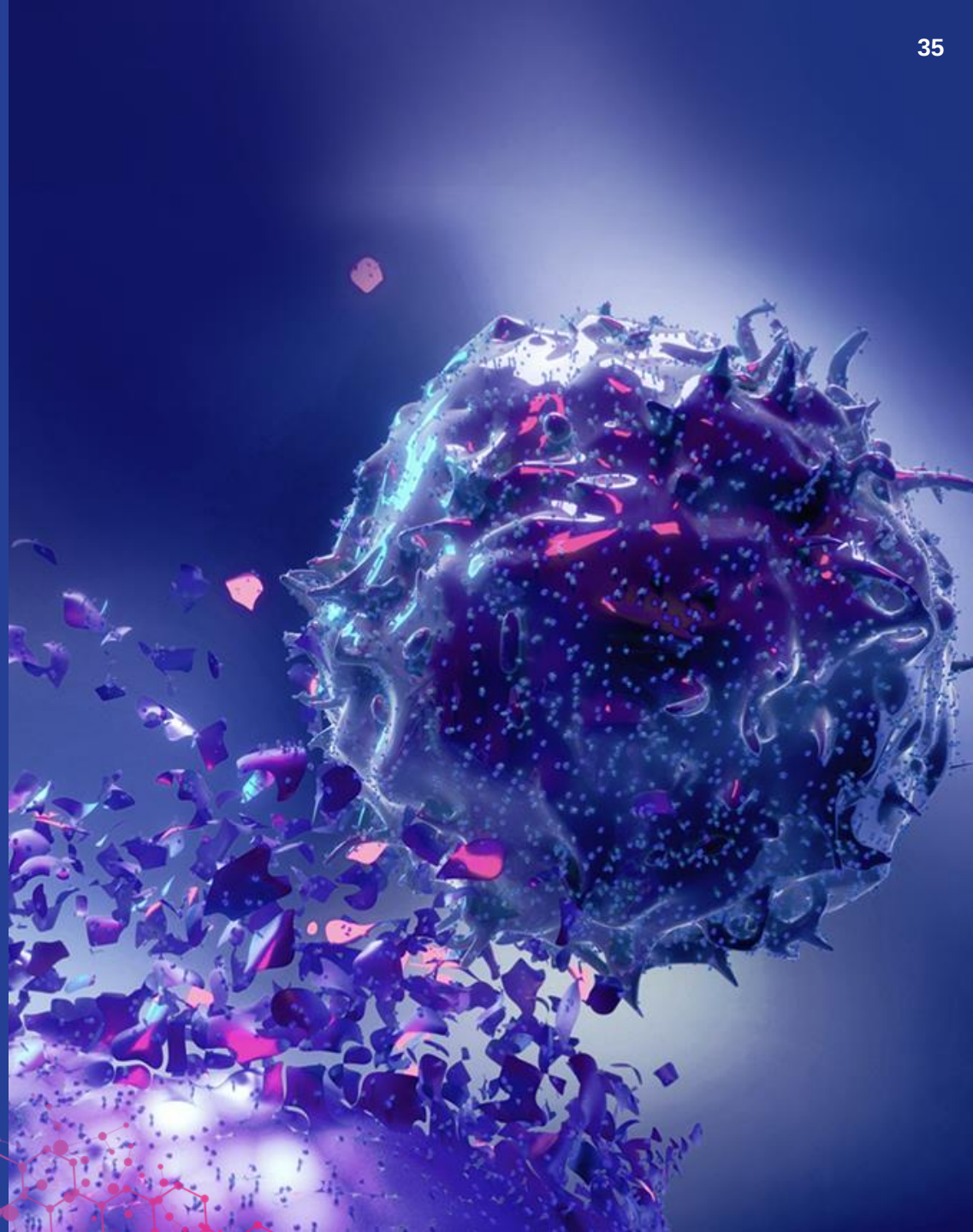
Immuno-Oncology

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[1]
- 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[2]
- 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.

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.

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Immuno-Oncology Clinical Development

Compound	Proposed Indication	Preclinical	Phase 1	Phase 2	Phase 3	Expected Upcoming Milestone	Collaborator
BXCL701[1] **Company-sponsored trials***	Small Cell Neuroendocrine Prostate Cancer (SCNC)					FDA Meeting	13 centers US / UK
	Small Cell Lung Cancer (SCLC)					Initiate Phase 1b/2	
BXCL701 **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
BXCL702[1] **BXCL701 follow-on/ novel DPP inhibitor***	Solid Tumors					Candidate nomination	

As of October 15, 2024

[1] Development paused due to Strategic Reprioritization announced on August 14, 2023

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

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Thank you!

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