



# Novel Treatment for Agitation in Neuropsychiatric Conditions

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March 2, 2023

# Forward-Looking Statements

This presentation includes "forward-looking statements" within the meaning of the Private Securities Litigation Reform Act of 1995. We intend 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 (the "Securities Act") and Section 21E of the Securities Exchange Act of 1934, as amended (the "Exchange Act"). 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, in particular BXCL501; potential benefits of treatment with BXCL501 and BXCL701; and potential market size and opportunity for product candidates. The words "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 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: the Company's 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 limited 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](http://www.sec.gov) and the Investors section of our 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 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](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@bioxcetherapeutics.com](mailto:medinfo@bioxcetherapeutics.com).

Please see full [Prescribing Information](#).

# Building a Unique Biopharmaceutical Business Model

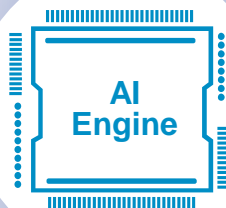
## Transformative Drug Re-innovation Approach Using AI

### BioXcel Therapeutics: Targeting High Unmet Needs in Neuroscience and Immuno-Oncology

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

### Neuroscience (BXCL501): First-in-human trials to FDA approval in just under 3.5 years

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



### OnkosXcel Therapeutics™

A subsidiary of BioXcel Therapeutics, Inc.

### Continuing Mission of Developing Transformative Medicine in Hard-to-Treat Tumors

### Lead Oncology Drug Candidate: BXCL701

- Unique oral innate immune activator, designed to turn cold tumors hot via DPP8/9 inhibition
- Combination approach, BXCL701 plus KEYTRUDA®
- Extend the value of IO in large underserved patient populations
- Focusing on cold tumor types

### High-Value Catalysts

- Positive Phase 2 data presented at ASCO GU, 2023
- SCNC Phase 2b trial initiation planned in 2023

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

\*\$15B Potential Market Opportunity<sup>1</sup>



## 139M Agitation Episodes<sup>1-3</sup>

**16M**  
BPD/SCZ  
Institutional  
Episodes<sup>1-3</sup>

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

**23M**  
BPD/SCZ  
At-Home\*  
Episodes<sup>1-3</sup>

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

**100M**  
Alzheimer's\*  
Episodes<sup>1</sup>

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

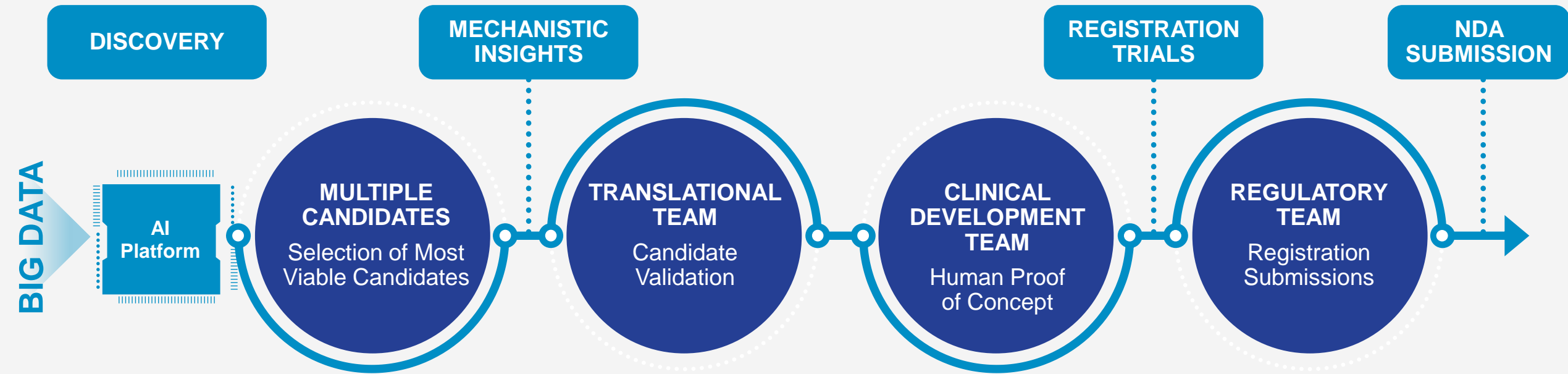
1. Data on file. BioXcel Therapeutics, Inc. New Haven, CT December 2020. 2. Wu EQ, Shi L, Birnbaum H, et al. Annual prevalence of diagnosed schizophrenia in the USA: a claims data analysis approach. Psychol Med. 2006;36(11):1535-1540. 3. National Institute of Mental Health. Prevalence of bipolar disorder 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

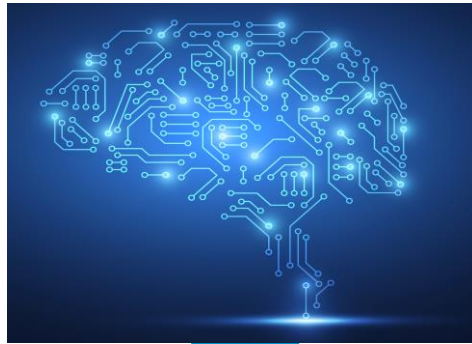
# Uniquely Integrated Drug Discovery & Development Capability

Utilizing Proprietary AI Platform



# Unique AI Approach: New Product Concepts with Focus on the Stress Axis

## STRESS



Stress alters the function and activity of CNS pathways

Changes in CNS pathway function and activity of CNS pathways

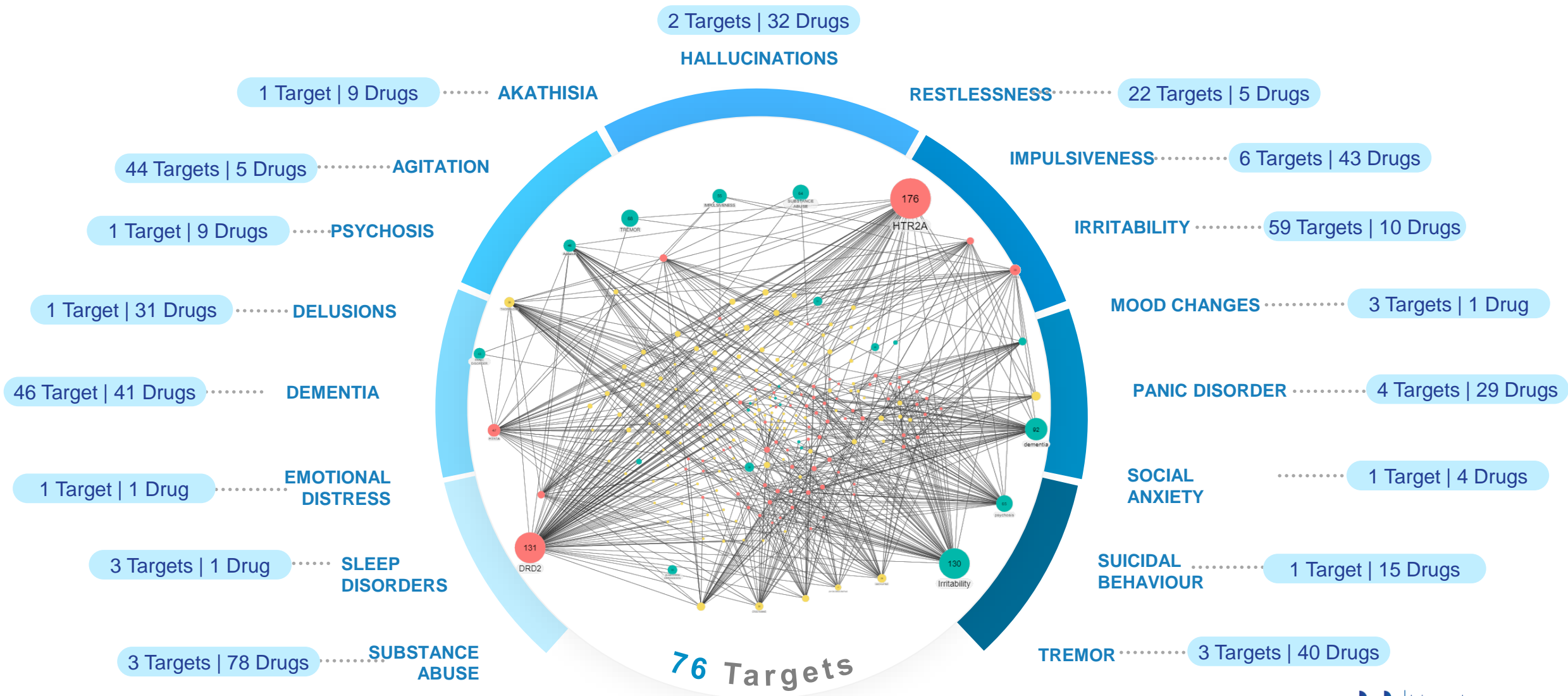


**MACHINE LEARNING CONNECTS:**

- Behaviors (symptoms)
- Stress pathways
- Drug targets
- Drugs

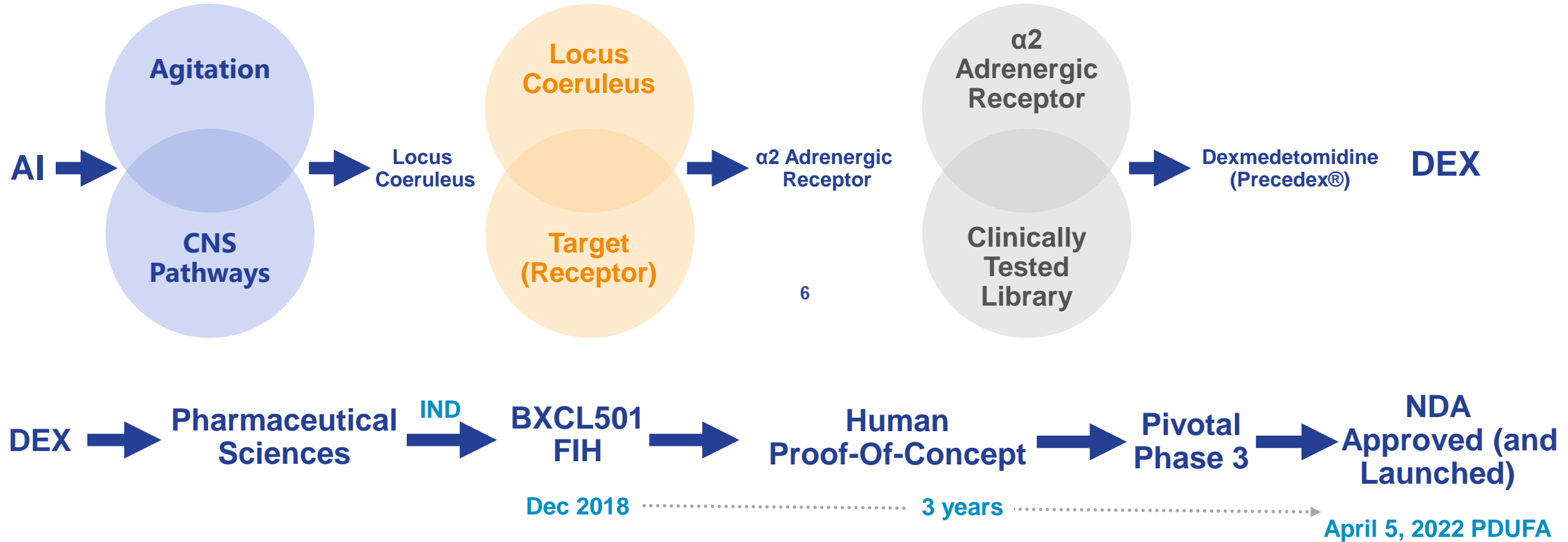
# Universe of Stress-related Symptoms, Targets & Drugs

## AI-Drive Insights Through Dynamic Connectivity Map





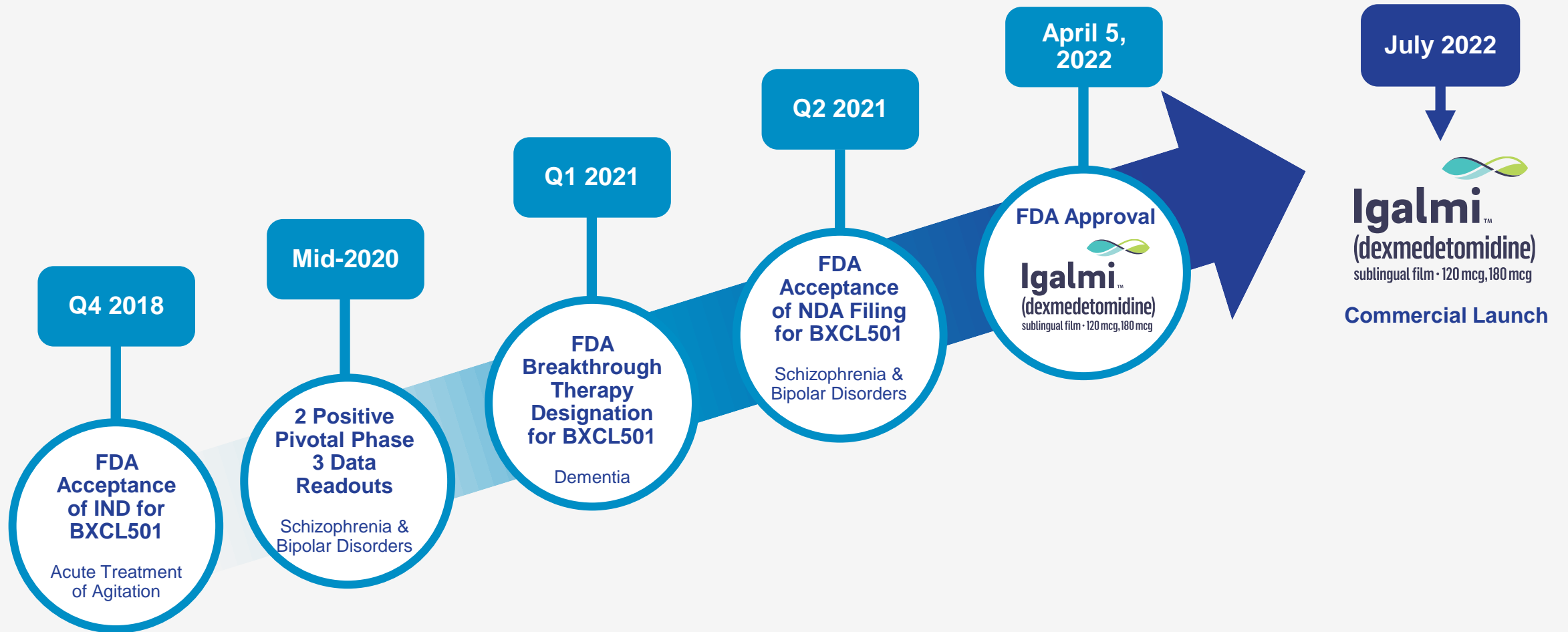
# BXCL501 AI-Enabled Development Journey



*Focus on discovery of novel pathways for stress-related symptoms*  
*Build sustainable, innovative pipeline of CNS drug candidates*

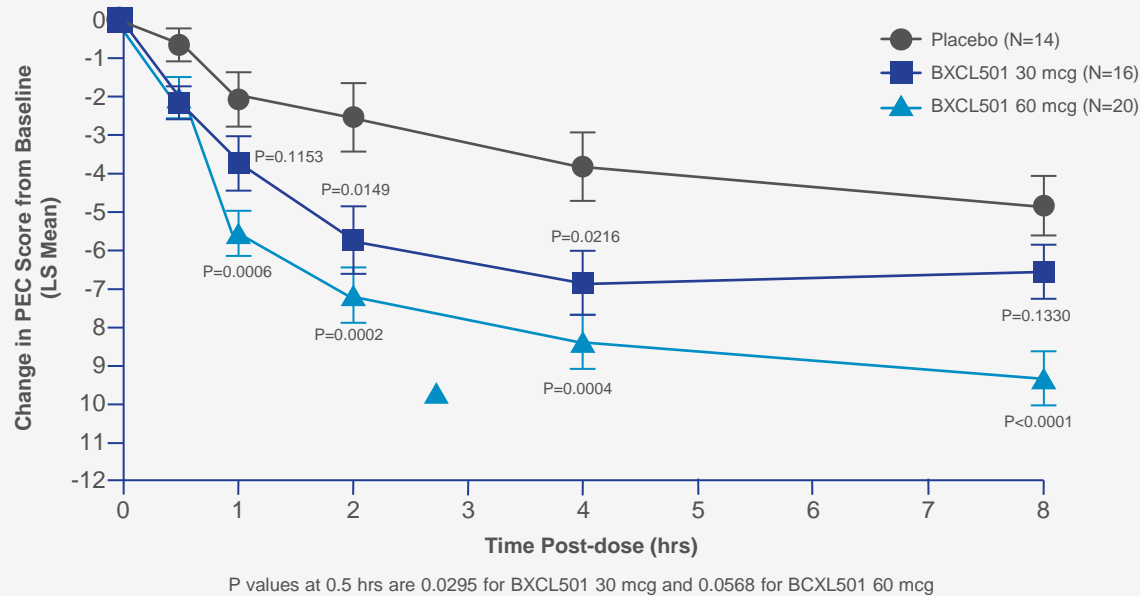
# IND to IGALMI™ Launch in 4 Years: Proven Business Model

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



# Clinically Meaningful, Rapid and Durable Response Observed

Change in PEC Score from Baseline



Placebo      BXCL501 30 mcg      BXCL501 60 mcg

Change from Baseline at 120 mins (LS Mean)

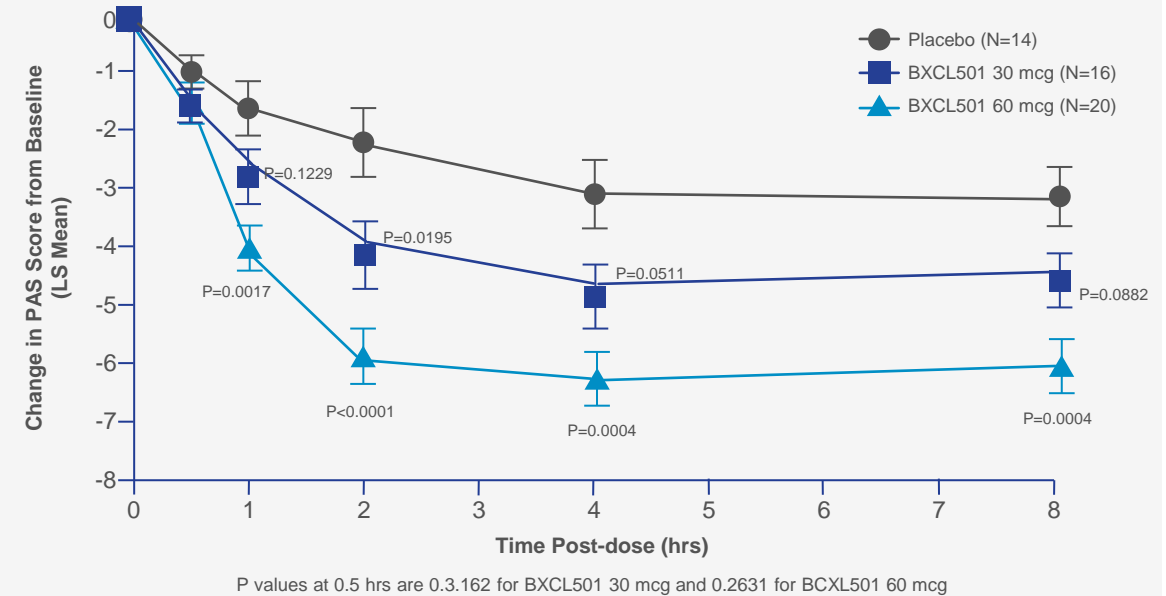
Placebo	-2.5	-5.7	-7.1
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Response °

Placebo	0%	31%	70%
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PANSS-Excitatory Component (PEC) is a 5 items scale: Excitement, Hostility, Tension, Uncooperativeness, Poor Impulse Control, rated 1-Absent to 7-Extreme  
 ITT analysis, Least Square Means ± SEM  
 ° Proportion achieving ≥ 40% PEC reduction

Change in PAS Score from Baseline



Placebo      BXCL501 30 mcg      BXCL501 60 mcg

Change from Baseline at 120 mins (LS Mean)

Placebo	-2.2	-4.1	-5.9
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Pittsburgh Agitation Scale (PAS) measures 4 behavior groups: aberrant vocalization, motor agitation, aggressiveness, and resisting to care rated 0- no agitation present to 4 – highest form of agitation.  
 ITT analysis, Least Square Means ± SEM

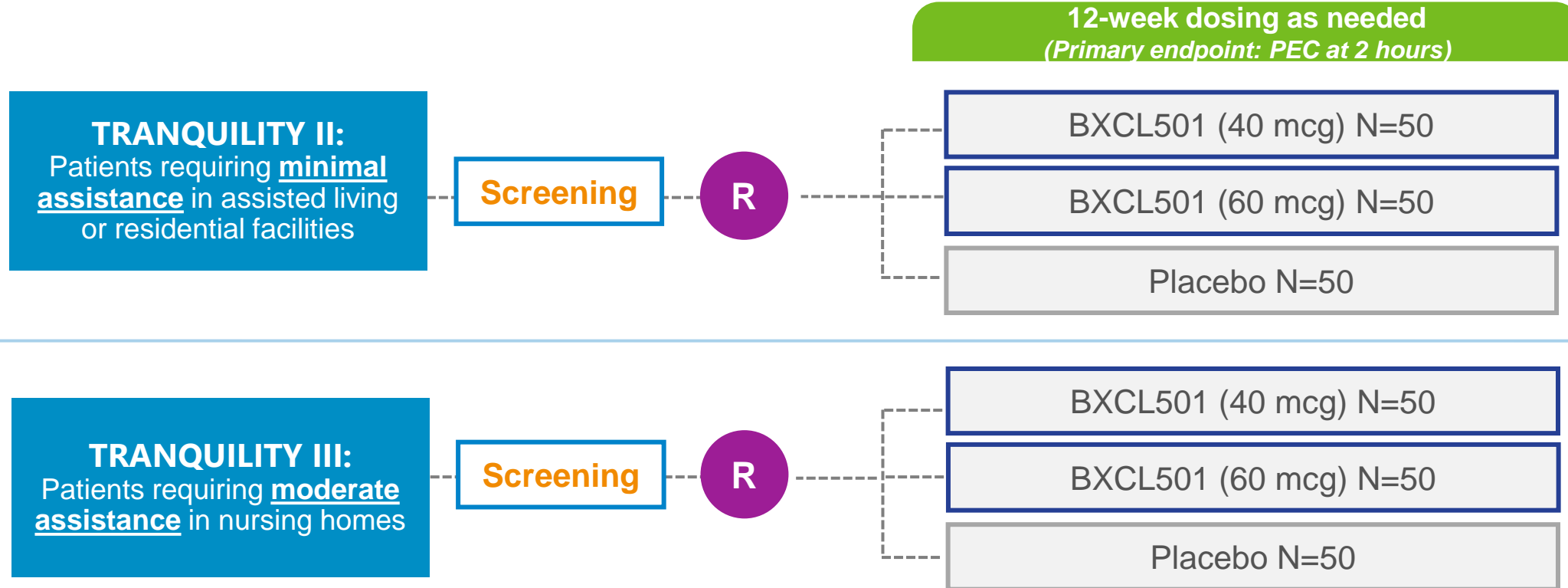
# BXCL501 Well Tolerated with No Severe or Serious Adverse Events

		BXCL501 30 mcg (N=16)	BXCL501 60 mcg (N=20)	Placebo (N=14)
Somnolence*	Mild	9 (56.3%)	11 (55.0 %)	0
	Moderate	0	1 (5.0 %)	
Hypotension	Mild	0 (0)	1 (5.0 %)	0
	Moderate	0 (0)	1 (5.0 %)	0
Orthostatic hypotension	Mild	0 (0)	1 (5.0 %)	0
	Moderate	1 (6.3 %)	0 (0)	0
Dizziness	Mild	1 (6.3 %)	1 (5.0 %)	0
	Moderate	0 (0)	0 (0)	
Bradycardia		0	1 (5.0 %)	0
Dry mouth		0	1 (5.0 %)	0
Nausea		0	1 (5.0 %)	0
Headache		0	1 (5.0 %)	0

\*Verbatim; drowsy or feeling sleepy

*All subjects self-administered the sublingual film*

# Initiated Pivotal Program for Acute Treatment of Agitation Associated With Alzheimer's Disease

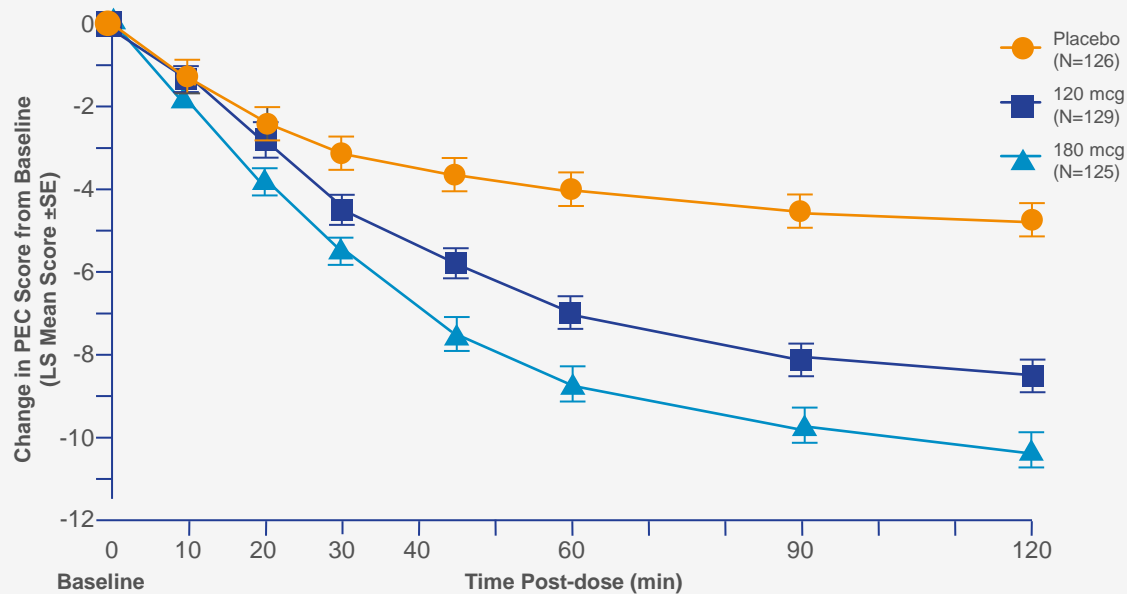


## Rollover Safety Study

Open-label, long-term, one-year safety study dosed as needed

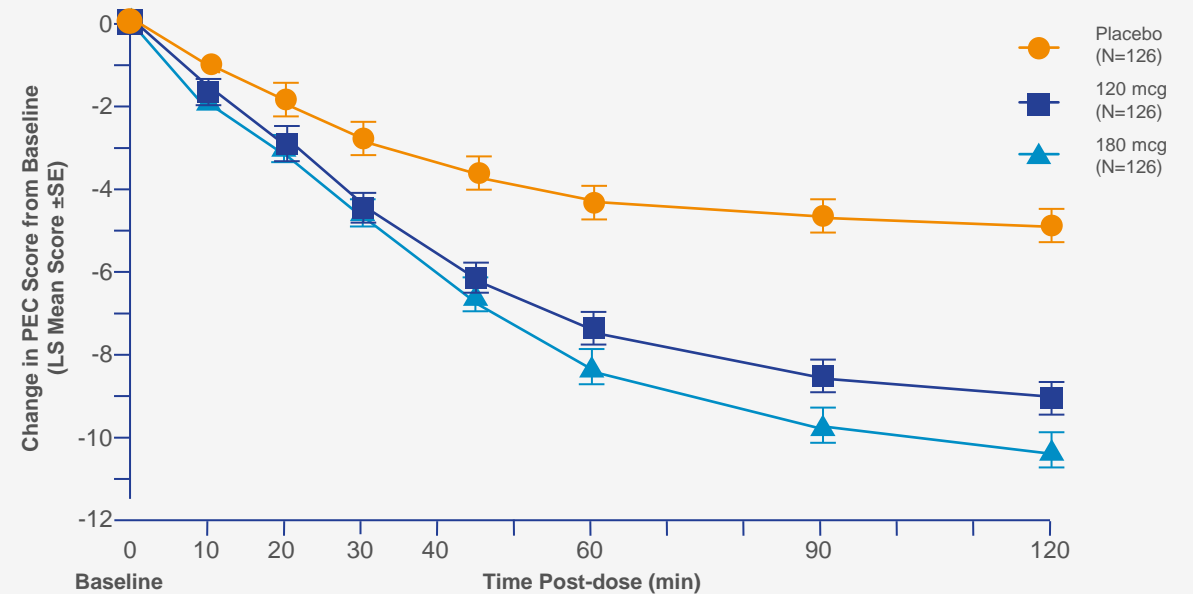
# Basis for IGALMI’s Final Label and Approval: SERENITY I and II Trials Demonstrated Onset of Action as Early as 20 Minutes and High Response Rate

**SERENITY I: Change in PEC Score from Baseline**



PEC baseline scores were 17.6, 17.5, and 17.6 in the placebo, IGALMI 120 mcg, and IGALMI 180 mcg groups, respectively.  
 •Note: Statistical significance vs. placebo was not observed until 30 minutes for the 120 mcg dose.

**SERENITY II: Change in PEC Score from Baseline**



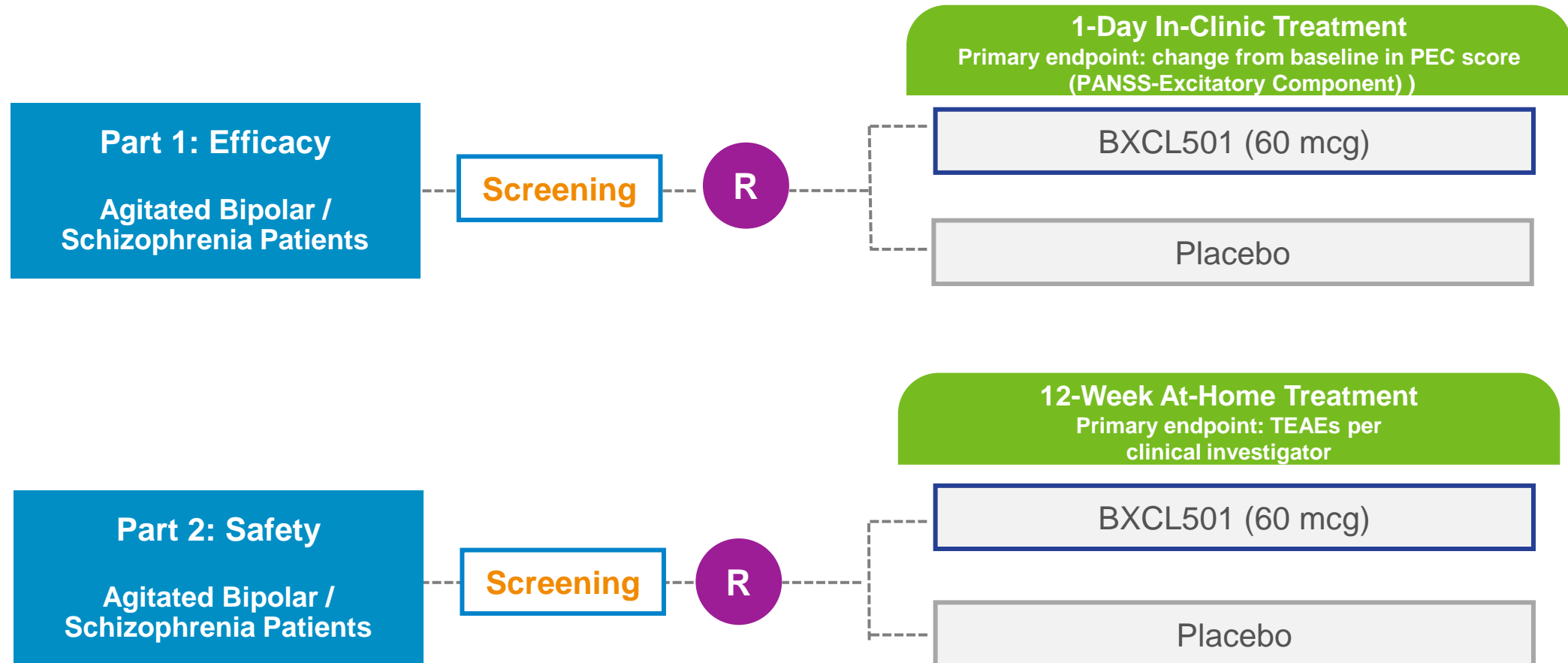
PEC baseline scores were 17.9, 18.0, and 18.0 in the placebo, IGALMI 120 mcg, and IGALMI 180 mcg groups, respectively.

**IGALMI met the primary and key secondary endpoint at the 120 mcg and 180 mcg doses, demonstrating statistically significant improvements from baseline**

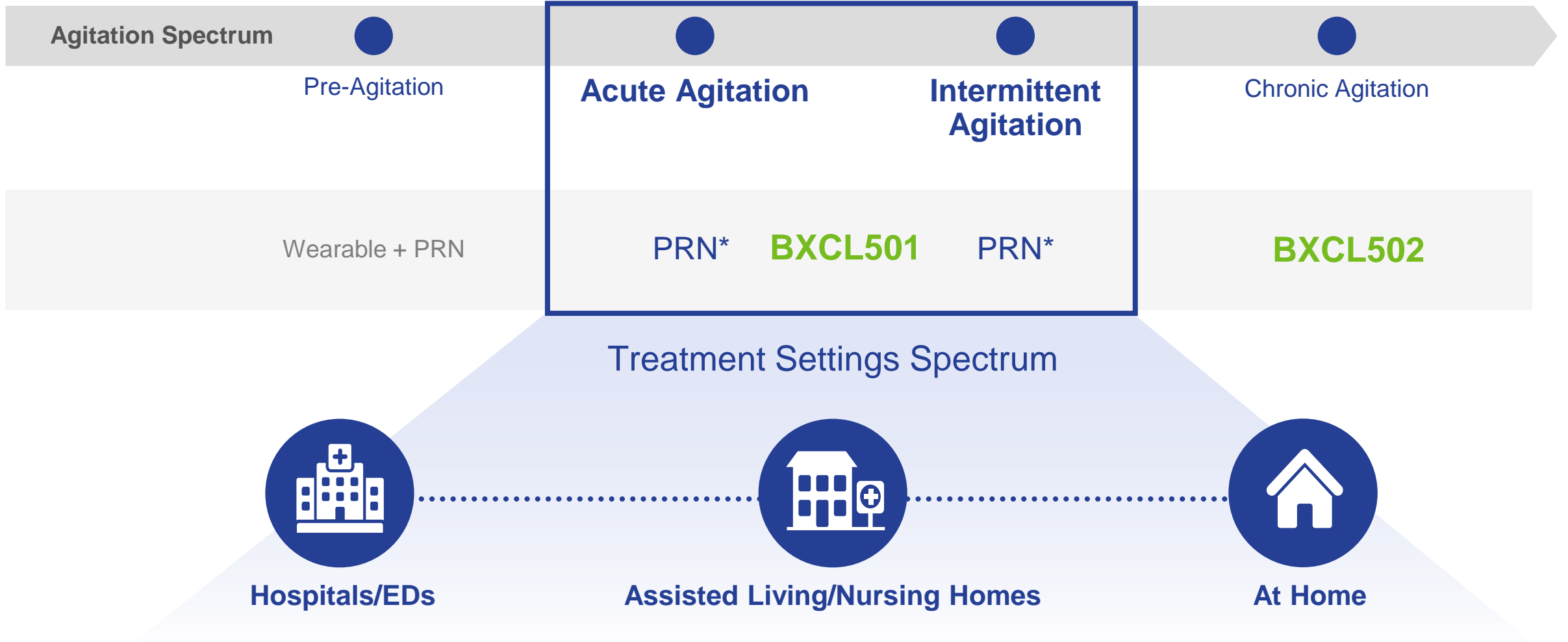
LS = least square; PEC = Positive and Negative Syndrome Scale – Excited Component; SE = standard error

# SERENITY III Pivotal Trial

At-home use of BXCL501 for Acute Treatment of Agitation in Bipolar and Schizophrenia Patients



# Comprehensive Alzheimer's Disease Program Strategy



\*As needed



# Thank you

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