NASDAQ: BTAI

Barclays Global Healthcare Conference

March 15, 2022

Forward-Looking Statements

This presentation includes "forward-looking statements" within the meaning of the Private Securities Litigation Reform Act of 1995. Forward-looking statements in this presentation include but are not limited to the advancement and development of BXCL501, BXCL502, and BXCL701, anticipated milestones, clinical development plans, the availability and results of data from clinical trials, expected patent terms and issuances, potential commercialization and related strategy and other information that is not historical information. When used herein, words including "anticipate," "being," "will," "plan," "may," "continue," and similar expressions are intended to identify forward-looking statements. In addition, any statements or information that refer to expectations, beliefs, plans, projections, objectives, performance, or other characterizations of future events or circumstances, including any underlying assumptions, are forward-looking. All forward-looking statements are based upon BioXcel Therapeutics' current expectations and various assumptions. BioXcel believes there is a reasonable basis for its expectations and beliefs, but they are inherently uncertain.

BioXcel Therapeutics may not realize its expectations, and its beliefs may not prove correct. Actual results could differ materially from those described or implied by such forward-looking statements as a result of various important factors, including, without limitation, its limited operating history; its incurrence of significant losses; its need for substantial additional funding and ability to raise capital when needed; its limited experience in drug discovery and drug development; its dependence on the success and commercialization of BXCL501 and BXCL701 and other product candidates; 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 BioXcel's product candidates; its approach to the discovery and development of product candidates based on EvolverAI is novel and unproven; 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 Annual Report on Form 10-K for the year ended December 31, 2021, as such factors may be updated from time to time in its other filings with the SEC, which are accessible on the SEC's website at **www.sec.gov** and the Investors section of our website at **www.bioxceltherapeutics.com**.

These and other important factors could cause actual results to differ materially from those indicated by the forward-looking statements made in this presentation. Any such forward-looking statements represent management's estimates as of the date of this presentation. While BioXcel Therapeutics may elect to update such forward-looking statements at some point in the future, except as required by law, it disclaims any obligation to do so, even if subsequent events cause our views to change. These forward-looking statements should not be relied upon as representing BioXcel Therapeutics' views as of any date subsequent to the date of this presentation.

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Our Mission

Develop transformative medicines utilizing AI approaches in neuroscience and immuno-oncology

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Neuroscience

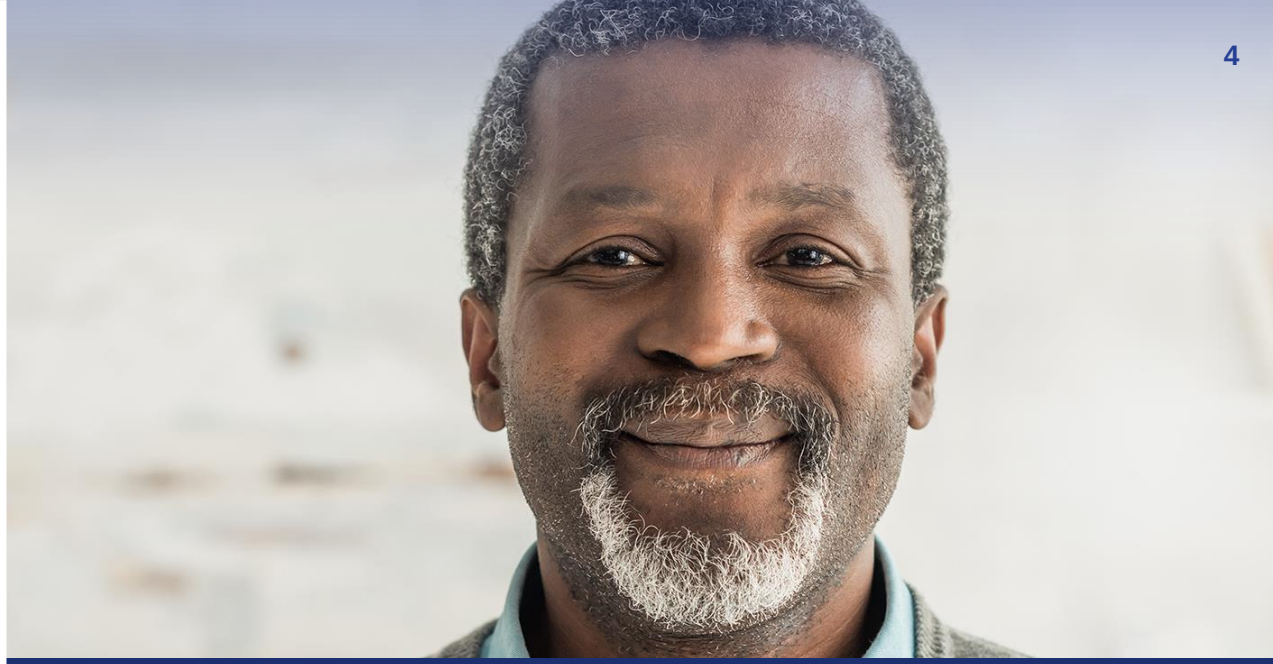
Symptoms from stress-related behaviors

BXCL501 Lead Program

- Schizophrenia related agitation
- Bipolar disorder related agitation
- Alzheimer’s disease related agitation
- Adjunctive treatment in major depressive disorder (MDD)

BXCL502 Pipeline Candidate

- Chronic agitation in Alzheimer’s disease



Immuno-Oncology

Innate Immunity

BXCL701 Lead Program

- Aggressive form of prostate cancer
- Advanced solid tumors

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Accelerating Drug Development through AI

The Value of AI-based Drug Development

- Optimizes R&D Economics
- Shortens Development Timelines
- Achieves Higher Probability of Success

AI-based approach can potentially impact neuroscience drug approval success rate
(compared to 6% industry approval rate)

Source: Biomedtracker and Pharmapremia 2020.

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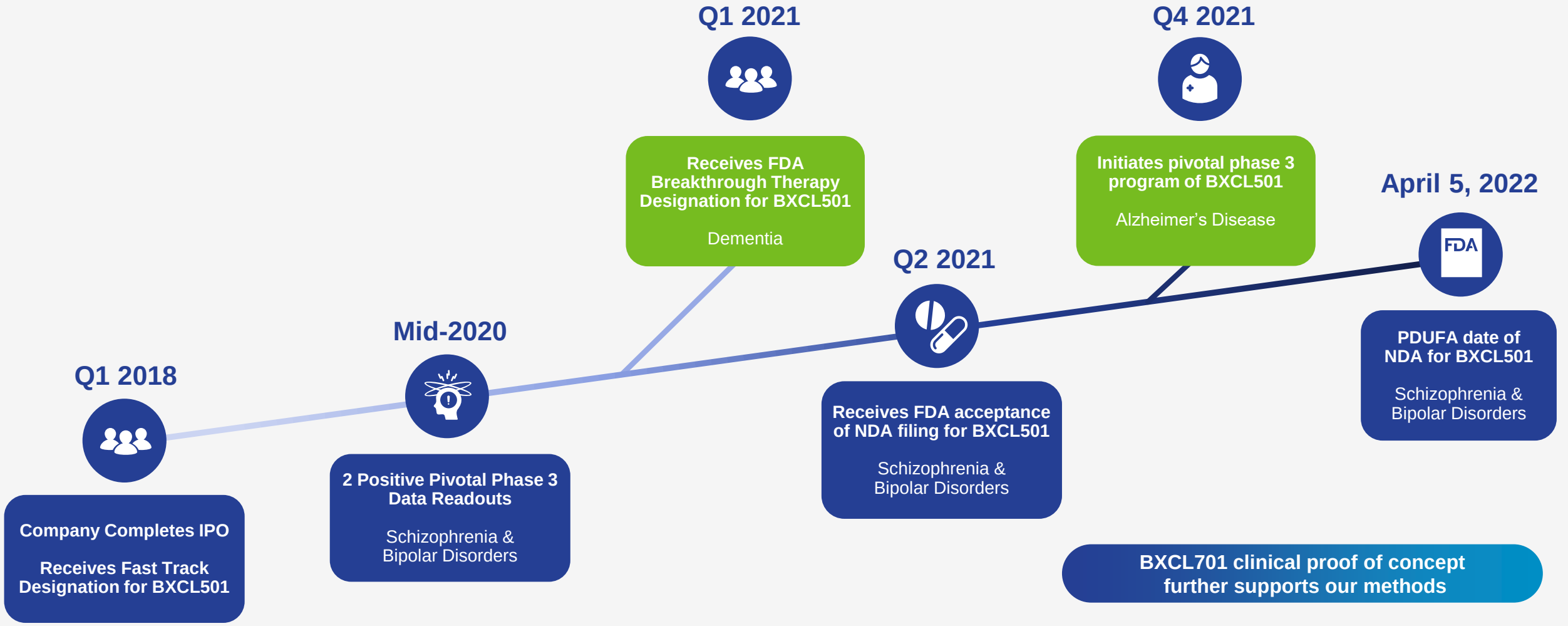
Our Rapid Journey to NDA for BXCL501

From first-in-human trial to acceptance of our NDA for BXCL501 in under 3 years



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Our Pipeline

Neuroscience

BXCL501	
Acute treatment of agitation associated with schizophrenia and bipolar disorders I and II	**SERENITY I & II Trials Completed (PDUFA date – 4/5/22)**
Acute treatment of agitation associated with Alzheimer's disease	**Pivotal Phase 3 Program Initiated**
Major depressive disorder (MDD)	**Ph1b/2 Trial Planned**
KalmPen™ (Single-use IM)	
Severe acute agitation	**Formulation Development**
BXCL502	
Chronic treatment of agitation in patients with dementia	**Formulation Development**
Wearable Device (+BXCL501)**	
Pre & post-agitation in dementia	**Feasibility Study Planned**

Immuno-oncology

BXCL701	
Metastatic castration-resistant prostate cancer (small cell neuroendocrine carcinoma and adenocarcinoma)	**Phase 1b/2 (Combination with KEYTRUDA®)**
Basket trial – hot and CPI resistant tumors (investigator-initiated study led by MD Anderson Cancer Center)	**Phase 2 (Combination with KEYTRUDA®)**

**Regulatory path to be determined; device + drug combination to be evaluated after further evaluation of predictive algorithm
Opioid withdrawal symptoms with BXCL501 pending NIDA grant decision
Agitation in delirium study with BXCL501 is on voluntary pause

Pipeline as of December 15, 2021

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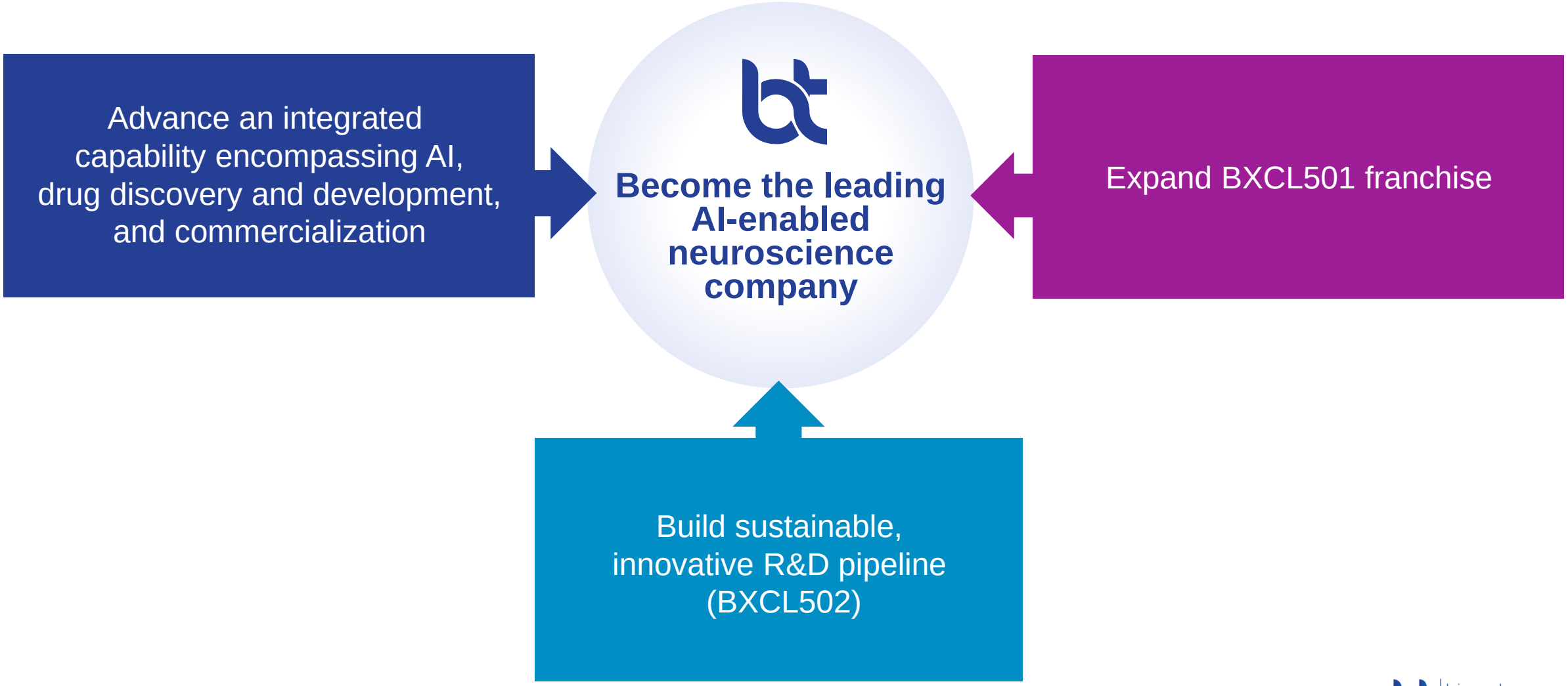
Five-Year Vision for Growth



Neuroscience Franchise

Acute treatment of agitation

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Agitation: Debilitating for Patients and Threatening for Healthcare Providers

A Common and Difficult-to-Manage Symptom

- A common occurrence in most neuropsychiatric disorders
- Characterized by recurring episodes requiring frequent treatments
- Over 150M people globally with schizophrenia, bipolar disorder, dementia, delirium and opioid use disorder[1]
- Over 13M patients in the U.S.[1] experience agitation within these disease areas
  - More than 200M agitation episodes per year in the U.S.[1]
  - Multi-billion-dollar healthcare burden
- Current treatment options are suboptimal
  - Physically restraining patients
  - Over-sedating therapies such as antipsychotics and benzodiazepines
  - Antipsychotic drugs have black box warnings for elderly
- BXCL501 has the potential to offer a novel mechanism and a highly differentiated approach

Proprietary, orally dissolving thin film formulation of dexmedetomidine (BXCL501)

[1]Internal company estimates

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Significant Commercial Potential in Multiple Indications

Schizophrenia & Bipolar Disorders

~ 7.3 million
estimated diagnosis in adult patients[1, 2]

~ 25M
agitation episodes per year based on diagnosed prevalence in the U.S.[1, 2]

Alzheimer's Disease

U.S. adults 65+ with AD
to **Double by 2040**[3]

From **5.8 million** (2020) to **11.8 million** (2040)

~100M
agitation episodes
per year in the U.S. [4]

Mental disorder drugs market size opportunity estimated to be up to $59B by 2031[5]

Sources: 1. Wu, 2006, NAMI 2. Prevalence of bipolar disorder in adults. November 2017. Accessed June 24, 2021. https://www.hcp.med.harvard.edu/ncs/ftpdir/NCS-R_12-month_Prevalence_Estimates.pdf 3 Alzheimer's Association .4 Estimate based on company market research 5 https://www.globenewswire.com/news-release/2021/06/11/2245922/0/en/Mental-Disorder-Drugs-Market-size-worth-US-58-91-Billion-by-2031-Visiongain-Research-Inc.html

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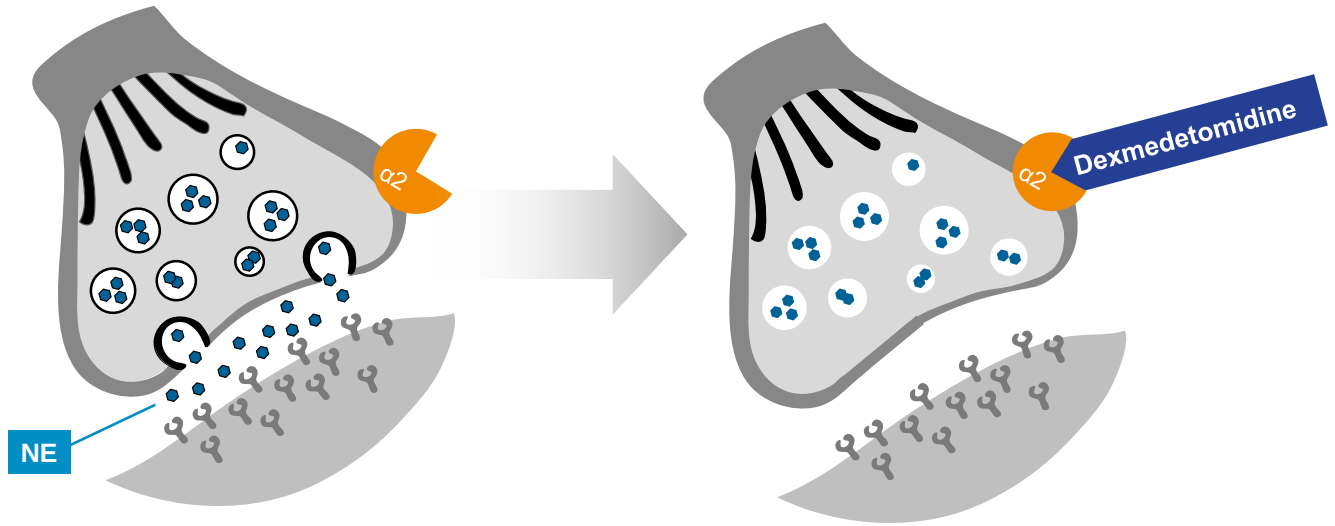
BXCL501: Novel Mechanism Potentially Targets Causal Agitation

Positive Results from Numerous Trials Support Underlying MoA

Dexmedetomidine MoA



(+) Agitation

(-) Agitation

Norepinephrine (NE)

Highly Differentiated from Current Treatments

- Easy-to-administer thin film, sublingual or buccal
- Non-invasive
- Non-traumatic
- Self-administered by patients

Expanding Patent Portfolio

- U.S. patent (No. 10,792,246) issued; IP protection expected until 2039
- Japanese patent (No. 6868698) directed to methods of treating agitation; expires no earlier than 2037
- Japanese design patent (No. 1681960) directed to film design; expires no earlier than 2045
- Multiple patent applications pending

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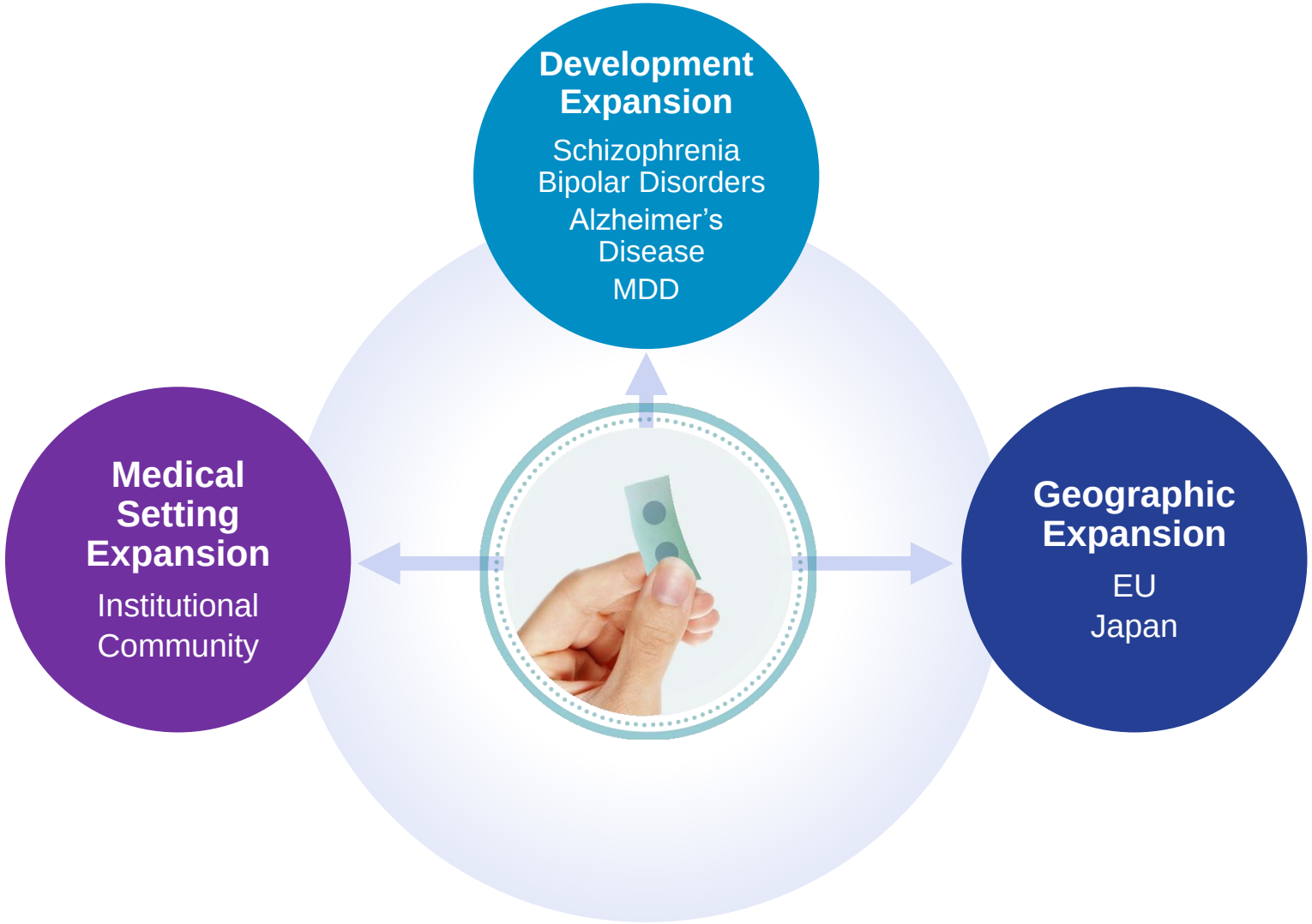
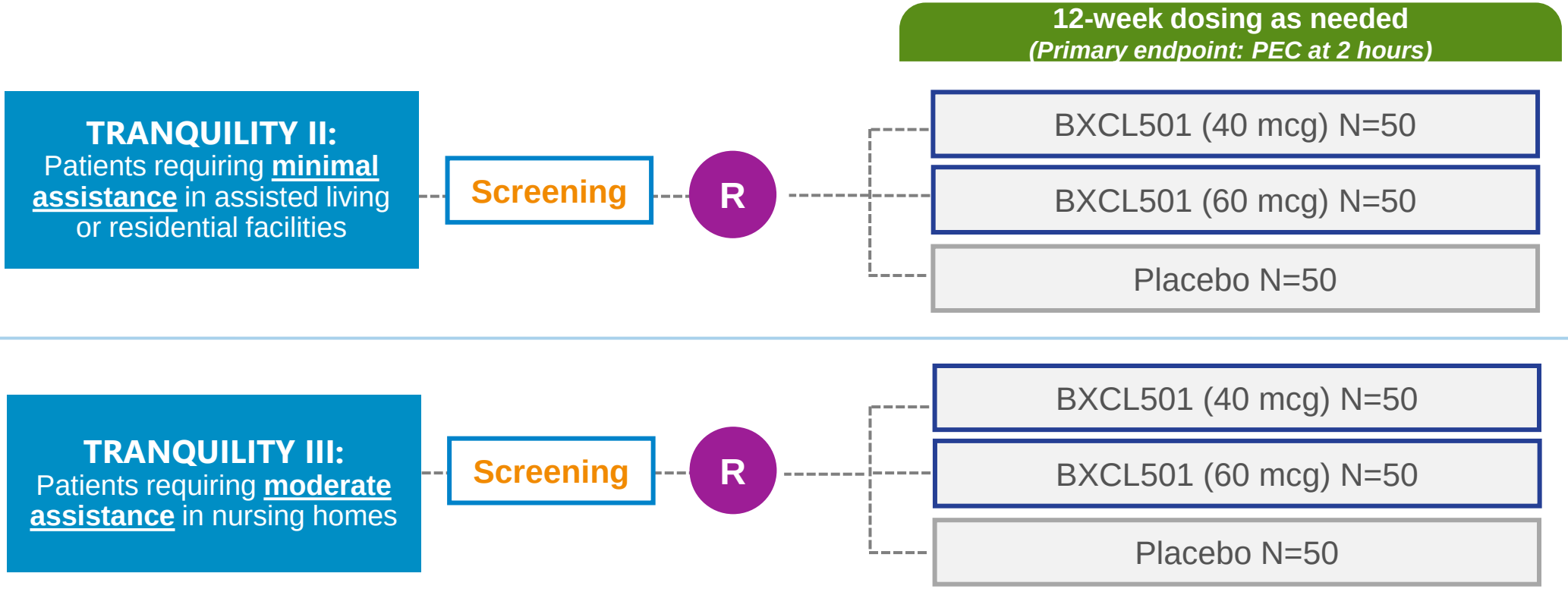
Considerable Portfolio Expansion Opportunity for BXCL501

Proprietary, Orally Dissolving Thin Film Formulation of Dexmedetomidine



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

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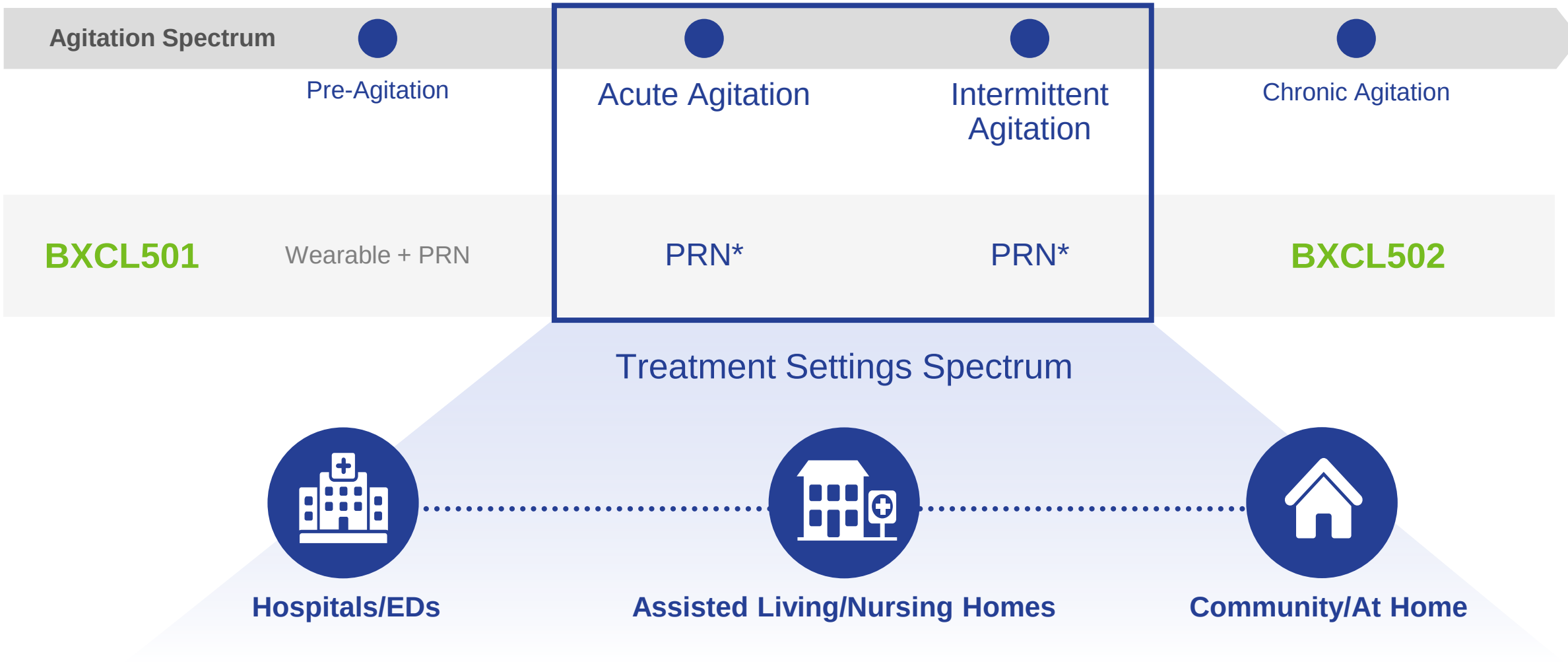
Alzheimer's Disease Program Comprehensive Strategy



*As needed

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BXCL501 Pharmacology Indicates a Direct Impact on Hard-to-Treat Depression Symptoms

- ✓ **Anxiety**
- ✓ **Restlessness**
- ✓ **Irritability**
- ✓ **Panic**
- ✓ **Sleep disturbances**
- Suicidality
- Sadness
- Concentration / Decision Making
- Diminished energy
- Anhedonia / Diminished interests
- Appetite
- Self worth
- Bodily symptoms
- Rejection sensitivity

BXCL501

During the first few weeks after initiation of SSRI/SNRI regimen, approximately 50% of patients are anxious, leading to poor compliance and clinical outcomes. **This represents a major opportunity for BXCL501.**

Preclinical and clinical data suggest BXCL501 will **rapidly treat** symptoms of depression that are not adequately treated by existing antidepressants.

Burden of Depression

300M+

Antidepressant prescriptions filled annually

Major limitation of slow onset and incomplete response

30M+

Americans currently prescribed antidepressants

12.7%

US Population over 12 years old took antidepressants in prior month

7%

12-month prevalence of Depression in US population

25%

Remain ill one year after starting treatment

Almost two-thirds are on antidepressants for >2 years

Sources: NIH/WHO, SAMHSA, NIMH
Pratt et al, 2017

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BXCL501 MDD Development Plan

Planned MAD Study

Healthy Volunteers

- Healthy volunteers, dosed daily
- **Objectives:** Assess safety, tolerability of daily doses of BXCL501

MDD Patients

- Depressed patients, dosed daily
- **Objectives:** Assess safety and tolerability in patients

Planned POC Trial in Depression

BXCL501 + SSRI

Placebo + SSRI

- Enroll patients with major depressive episode treated with SSRI or SNRI
- 4- to 6- week double blind, placebo-controlled parallel group trial
- **Objectives:** Assess antidepressant efficacy and safety of daily BXCL501

Phase 3 Pivotal Trial in Depression

- **Objectives:** Assess antidepressant efficacy and safety of daily BXCL501

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BXCL501

PDUFA date: April 5, 2022

Commercial and Launch Readiness

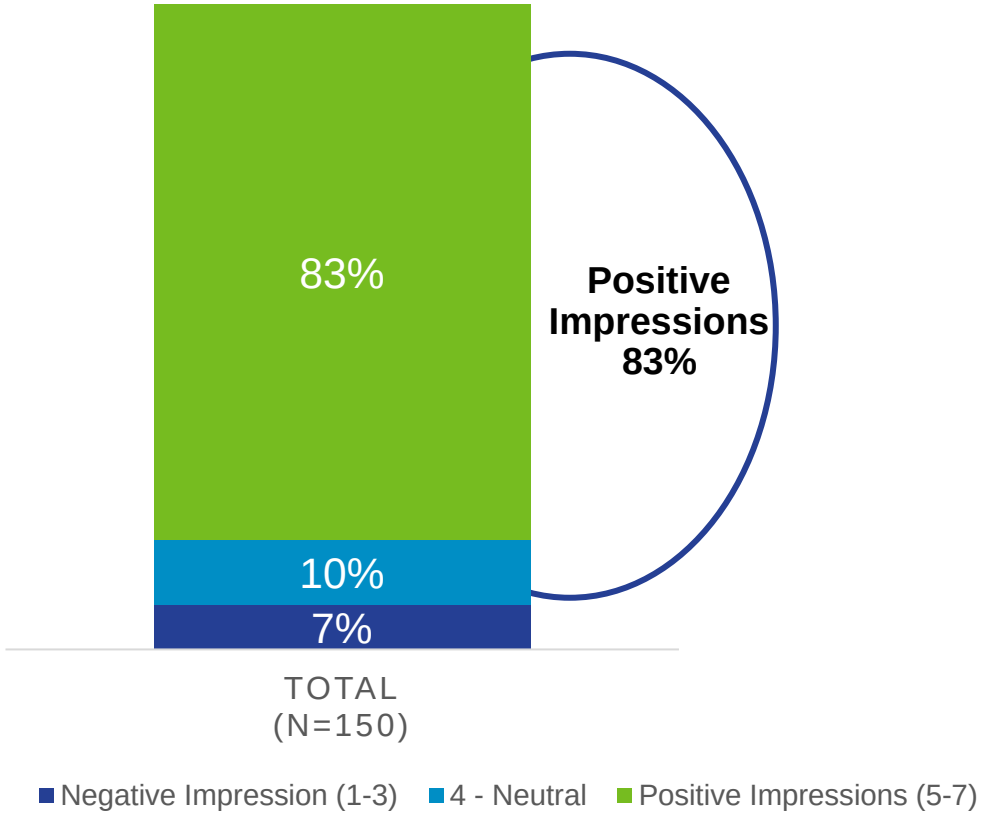
Informing and Educating Healthcare Professionals

- Fully deployed Medical Science Liaison and Medical Managed Care teams
- Actively engaged with healthcare professionals and payers
- Fully launched unbranded disease education campaign (Including partnersincalm website)
- Participating and presenting at leading conferences
- Submitted pivotal study manuscript for publication

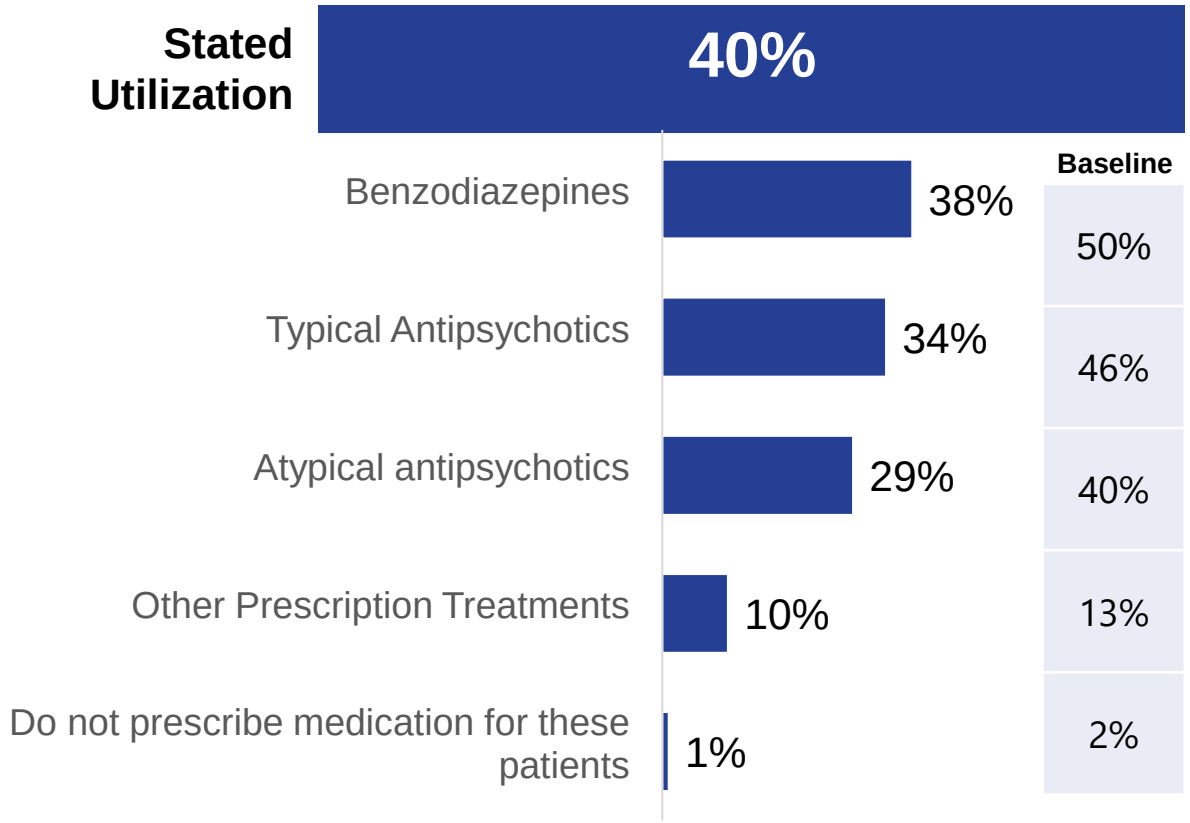
83% of HCPs surveyed have a positive impression of BXCL501*

Would consider prescribing for 40% of their accessible acute agitation population

Overall Impression



HCP-Stated Utilization



*HCP Quantitative Detail Aid Research (n=150), January 2022

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Ready for Potential Launch

- Generating market insights through quantitative research
- Optimizing market access and pricing strategy for BXCL501 through evidence-based market research
- Finalizing promotional materials based on strong research insights
- Hired Chief Commercial Officer, Matt Wiley
- Expanded sales leadership and building a national sales team

Immuno-oncology Franchise

BXCL701: Differentiated First-in-Class Oral Innate Immune Activator

Candidate	• One of most advanced **orally available** innate activators in the clinic • **De-risked:** single agent activity + large safety database

Function / MoA Biomarker	• Designed to:
	  • Mediate increase in key pro-inflammatory **cytokines**
	  • Activate **inflammasome** via DPP 8/9
	• Indications chosen based on frequency of DPP mutations

Clinical Efficacy	• Pro-inflammatory activity inflames tumor microenvironment and is designed to:
	  · **Augment and deepen responses** in checkpoint inhibitor naïve patients
	  · **Reverse resistance** in patients who have progressed on checkpoint inhibitor
	  · **Extend activity** into cold tumors

Indications	• Metastatic castration-resistant prostate cancer — adenocarcinoma and small-cell/neuroendocrine • Relapsed Solid Tumors (Hot Tumors)
External Validation	GILEAD, Forty Seven, Pfizer, TRILLIUM THERAPEUTICS INC.

Robust AI Platform
nature chemical biology
Validation of AI Approach[1]

Innate Immunity Activator
Journal for ImmunoTherapy of Cancer
Mechanism of action[2]

Orphan Drug Designations
FDA
Pancreatic Cancer
Melanoma
AML
Sarcoma

Clinical Partnership
THE UNIVERSITY OF TEXAS MD Anderson Cancer Center

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1. Nature Chemical Biology, volume 13, pages 46–53 (2017)
2. Journal for ImmunoTherapy of Cancer 2021; 9:e002837. doi:10.1136/jitc-2021-002837"

Clinical Proof of Concept for BXCL701

ASCO Genitourinary Cancers Symposium 2022

In heavily pre-treated mCRPC patient population

- 93% enrolled SCNC patients pre-treated with platinum
- All enrolled adenocarcinoma patients pre-treated with ≥1 line of TAXANE chemotherapy and 59% with 2 androgen signaling inhibitors

BXCL701 + KEYTRUDA pembrolizumab demonstrated manageable safety profile

- Majority of AEs were low grade
- No evidence of potentiation of immune-related AEs

NEPC 33% composite response rate (n = 15)

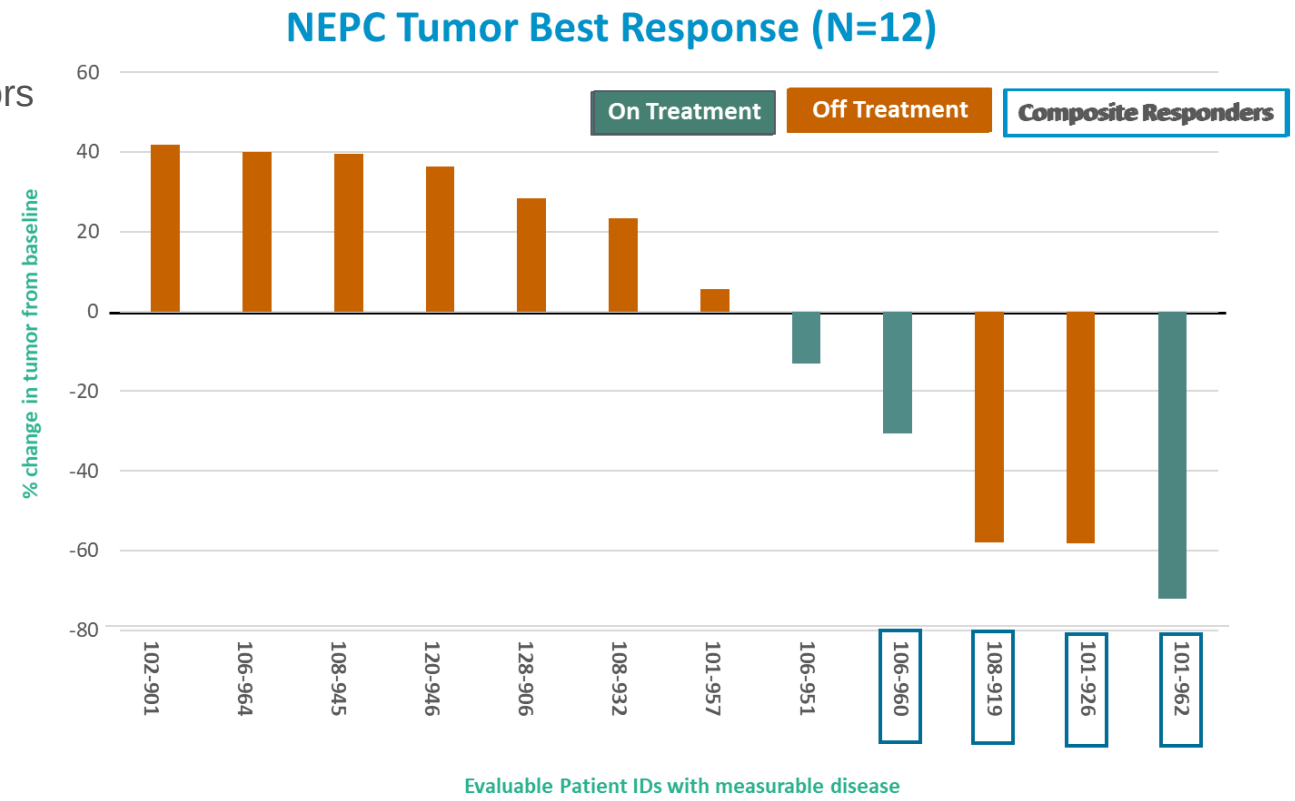
- 33% RECIST-defined PR
- 58% disease control rate (CR + PR + SD)

Adenocarcinoma 21% composite response rate (n = 29)

- 22% RECIST-defined PR
- 83% disease control rate (CR + PR + SD)
- 17% PSA_{50} including 5 patients with -100% to -57% PSA drop

KEYTRUDA single agent historic data[1]*

- Objective response rate 3-5%
- Disease control rate 12%
- PSA_{50} response 6%



[1] Antonarakis et al. "Pembrolizumab for Treatment-Refractory Metastatic Castration-Resistant Prostate Cancer: Multicohort, Open-Label Phase II KEYNOTE-199 Study." *Journal of Clinical Oncology* 38, no. 5 (February 10, 2020) 395-405. DOI: 10.1200/JCO.19.01638.

**FOR ILLUSTRATIVE PURPOSES ONLY: no head-to-head studies have been conducted comparing BXCL701 to pembrolizumab as a single agent. Differences exist between trial designs and subject characteristics, and caution should be exercised when comparing data across studies.*

Oral BXCL701 + pembrolizumab demonstrated encouraging anti-tumor activity in heavily pre-treated, refractory mCRPC patients with SCNC and adenocarcinoma phenotypes

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Exciting Year Ahead

2022 Catalysts: Intersection of Innovation & Commercialization

Strong cash position ~ $233M* to fund key milestones**

NEUROSCIENCE: BXCL501

Schizophrenia & Bipolar Disorders

- ✓ PDUFA date of April 5, 2022
- ✓ MAA submission to EMA anticipated in 1H 2022
- ✓ Seeking partner in Europe

Alzheimer's Disease***

- ✓ Pivotal phase 3 program underway

Major Depressive Disorder

- ✓ Meeting with FDA held in Q4 2021 on key design features of MDD program

COMMERCIAL LAUNCH READINESS: BXCL501

- ✓ Ready to launch upon approval in U.S.

IMMUNO-ONCOLOGY: BXCL701

Aggressive Form of Prostate Cancer (cold tumor)

- ✓ Announced additional efficacy and safety data in adenocarcinoma and neuroendocrine prostate cancer Phase 1b/2 trial presented at ASCO Genitourinary Cancers Symposium in February 2022

Relapsed Solid Tumors (hot tumors)

- ✓ Interim efficacy data readout expected in 2H 2022

*Cash and cash equivalents as of Dec. 31, 2021
**Pipeline as of Dec. 15, 2021
***Acute treatment of agitation in dementia patients with Alzheimer's disease

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Thank you!

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