



40th Annual J.P. Morgan Healthcare Conference

January 13, 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 Quarterly Report on Form 10-Q for the quarter ended September 30, 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.

This presentation includes information about the Company's estimated cash position as of December 31, 2021. The estimate is unaudited and preliminary and does not present all information necessary for an understanding of the Company's financial condition as of December 31, 2021 and its results of operations for the three months and year ended December 31, 2021. The completion of the Company's year-end accounting procedures, including execution of the Company's internal control over financial reporting, and audit of the Company's financial statements for the year ended December 31, 2021 by the Company's independent registered public accounting firm is ongoing and could result in changes to this preliminary information.

Our Mission

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





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



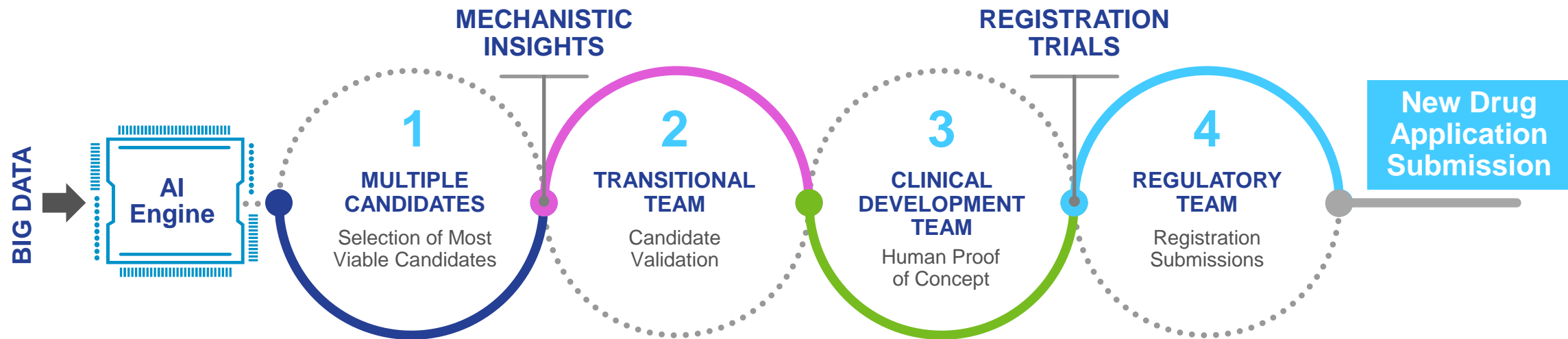
Immuno-Oncology

Innate Immunity

BXCL701 Lead Program

- Aggressive form of prostate cancer
- Advanced solid tumors

Accelerating Drug Development through AI



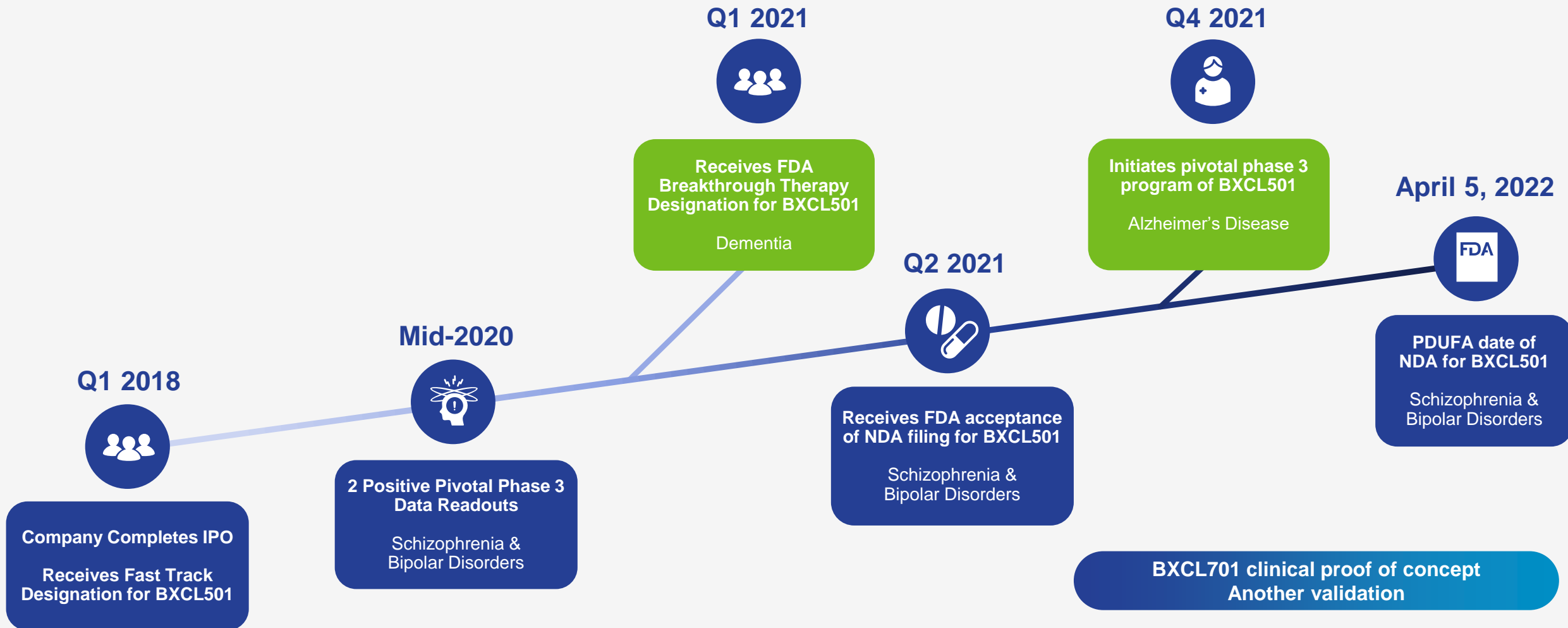
The Value of AI-based Drug Development

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

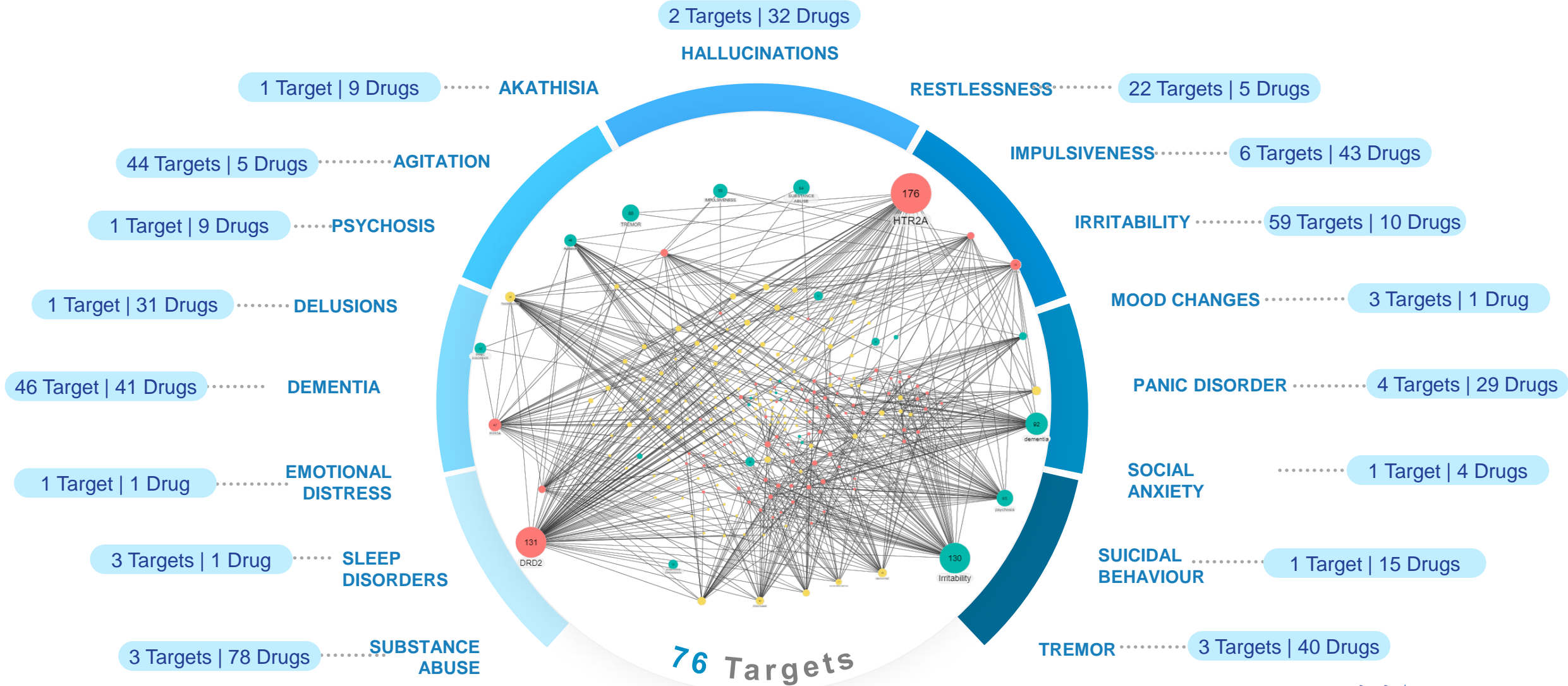
**AI-based approach can potentially impact neuroscience drug approval success rate
(compared to 6% industry approval rate)**

Our Rapid Journey to NDA for BXCL501

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



Universe of Stress-related Symptoms, Targets & Drugs: Dynamic AI Connectivity Map to Identify Potential Candidates



Our Pipeline

Neuroscience

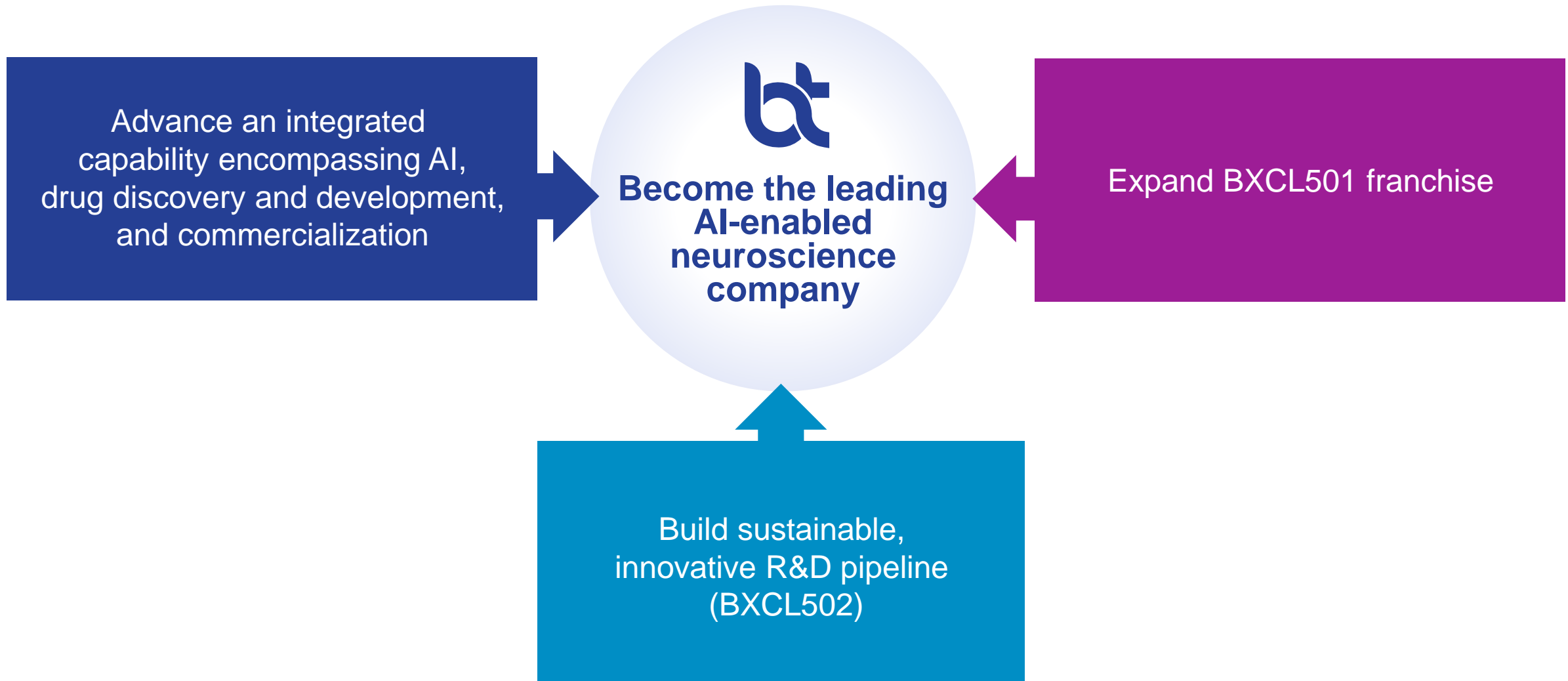
BXCL501	
Acute treatment of agitation associated with schizophrenia and bipolar disorders I and II	SERENITY I & II Trials Completed (PDUFA date – 4/5/22)
Acute treatment of agitation associated with Alzheimer's disease	Pivotal Phase 3 Program Initiated
Major depressive disorder (MDD)	Ph1b/2 Trial Planned
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

Five-Year Vision for Growth



Neuroscience Franchise

Acute treatment of agitation

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 disorder¹
- 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)*

¹Internal company estimates

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 AD²
to **Double by 2040**³

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

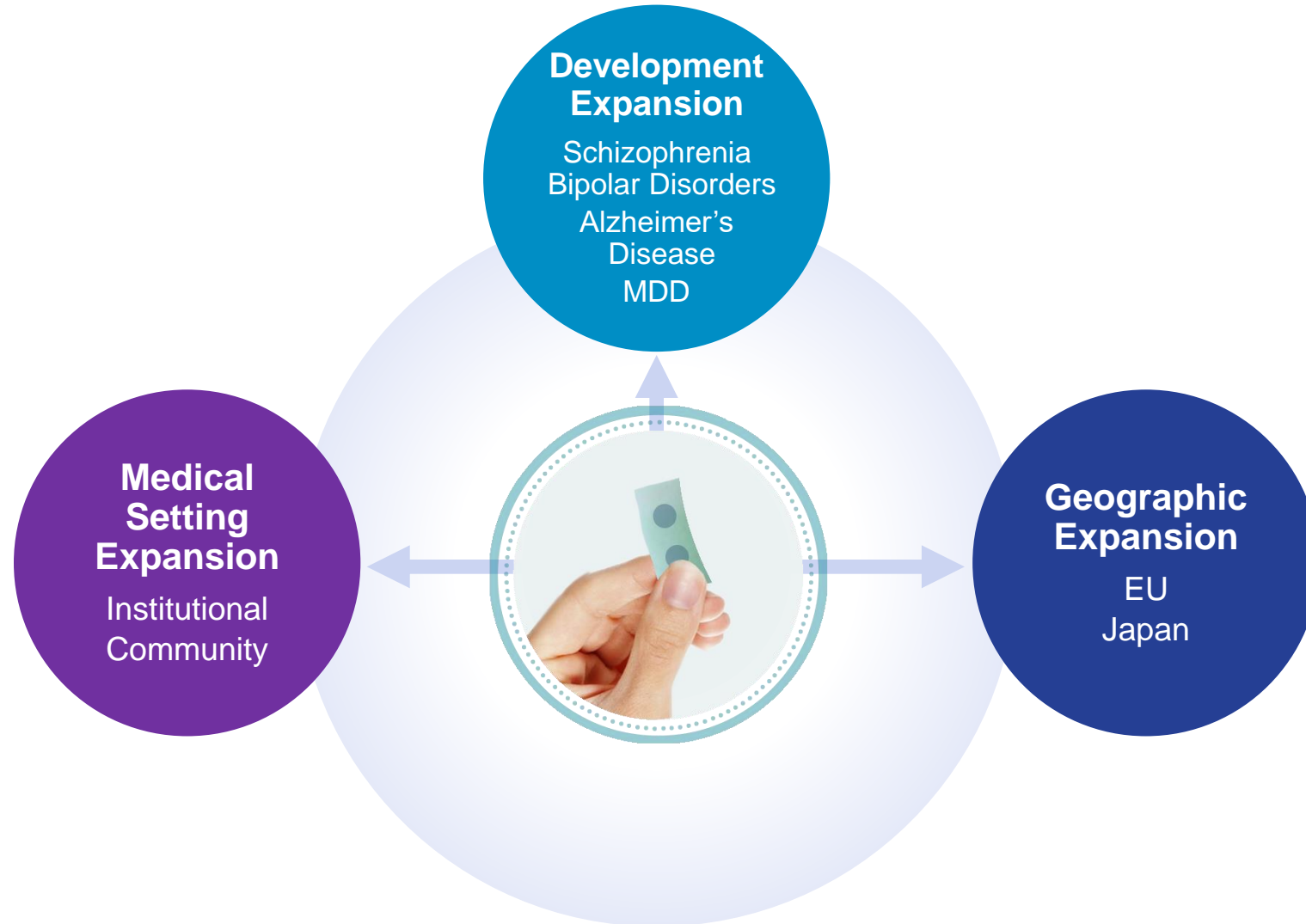
~100M
agitation episodes
per year in the U.S.⁴

Mental disorder drugs market size opportunity estimated up to \$59B by 2031⁵

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>

Considerable Portfolio Expansion Opportunity for BXCL501

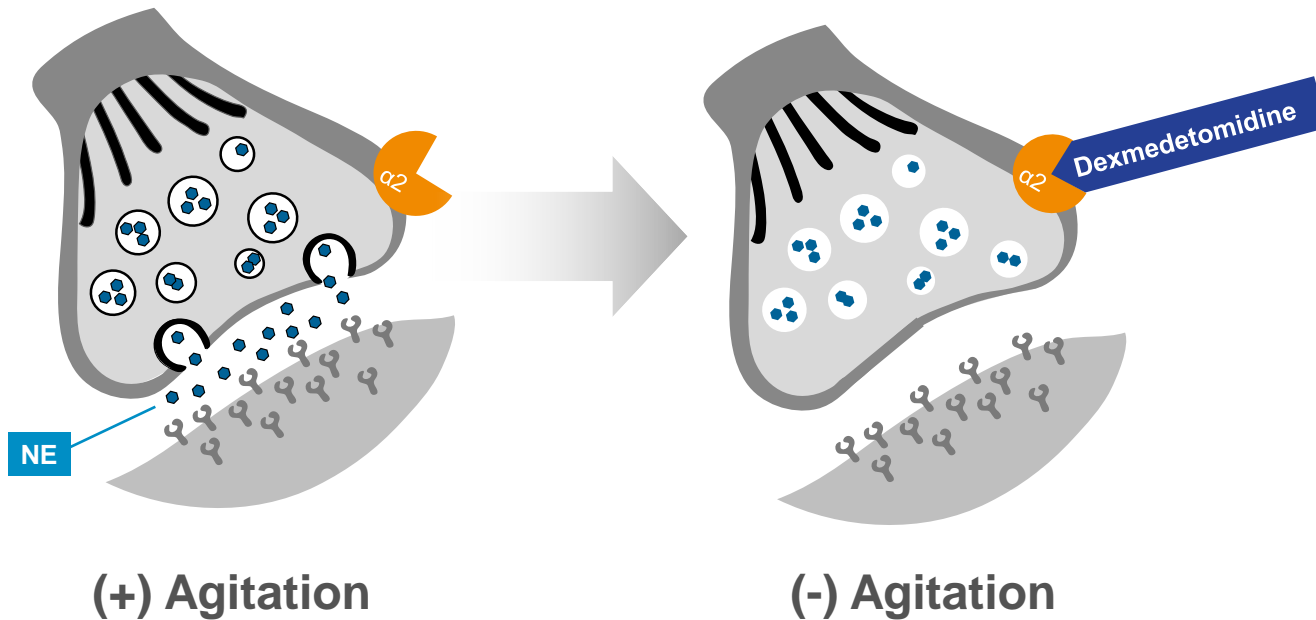
Proprietary, Orally Dissolving Thin Film Formulation of Dexmedetomidine



BXCL501: Novel Mechanism Potentially Targets Causal Agitation

Positive Results from Numerous Trials Support Underlying MoA

Dexmedetomidine MoA



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

Positive Results Observed in Patients with Schizophrenia and Bipolar Disorders

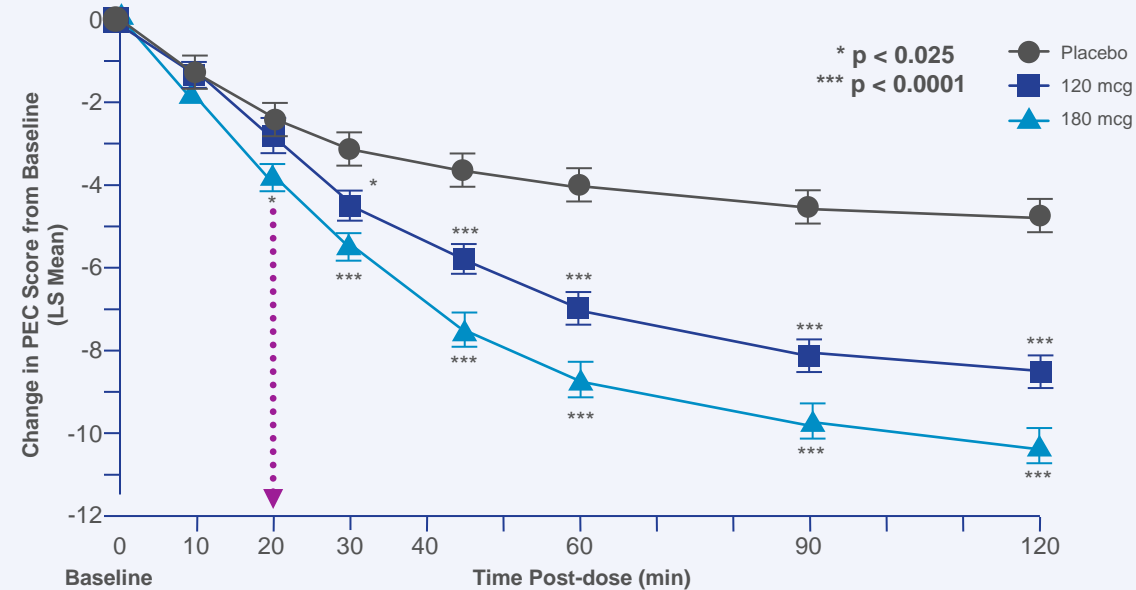


FDA Fast Track Designation

for the acute treatment of agitation associated with schizophrenia, bipolar disorders and Alzheimer's disease

Clinically meaningful, rapid and durable reductions in agitation observed

SERENITY I: Change in PEC Score from Baseline



PDUFA date of April 5, 2022

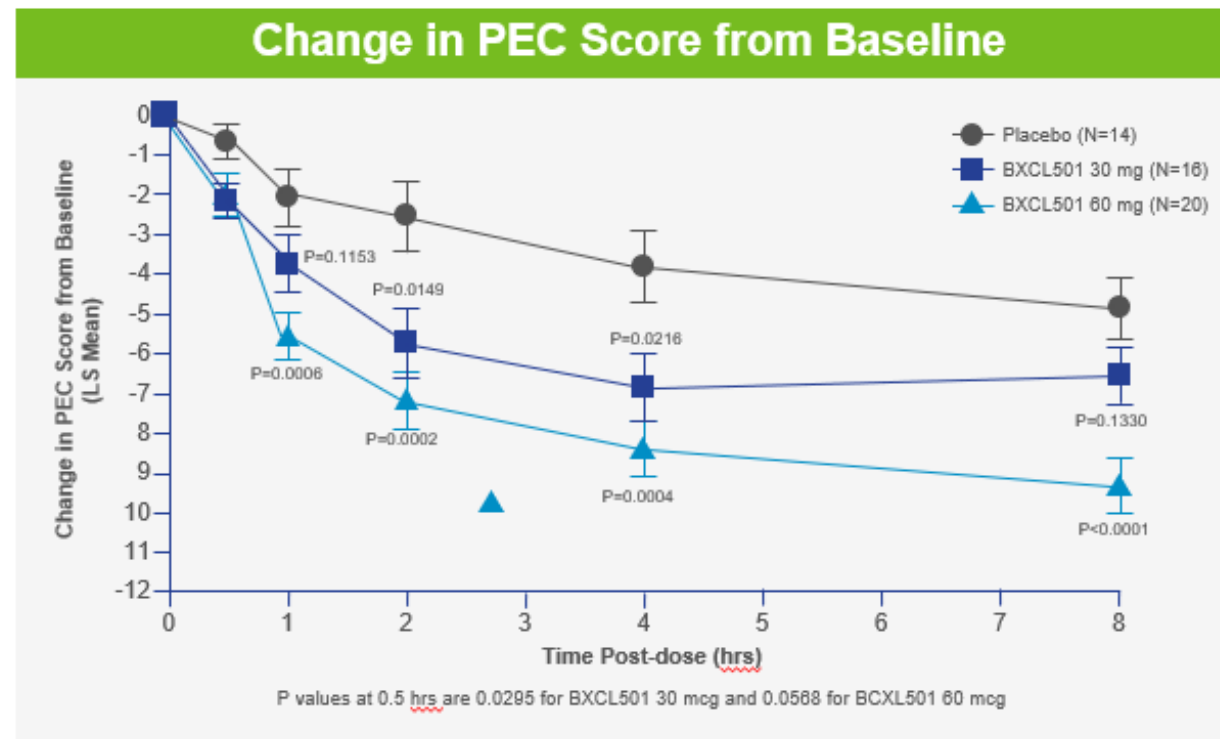
Significant Improvement Demonstrated in Dementia-Related Agitation



FDA Breakthrough Therapy Designation

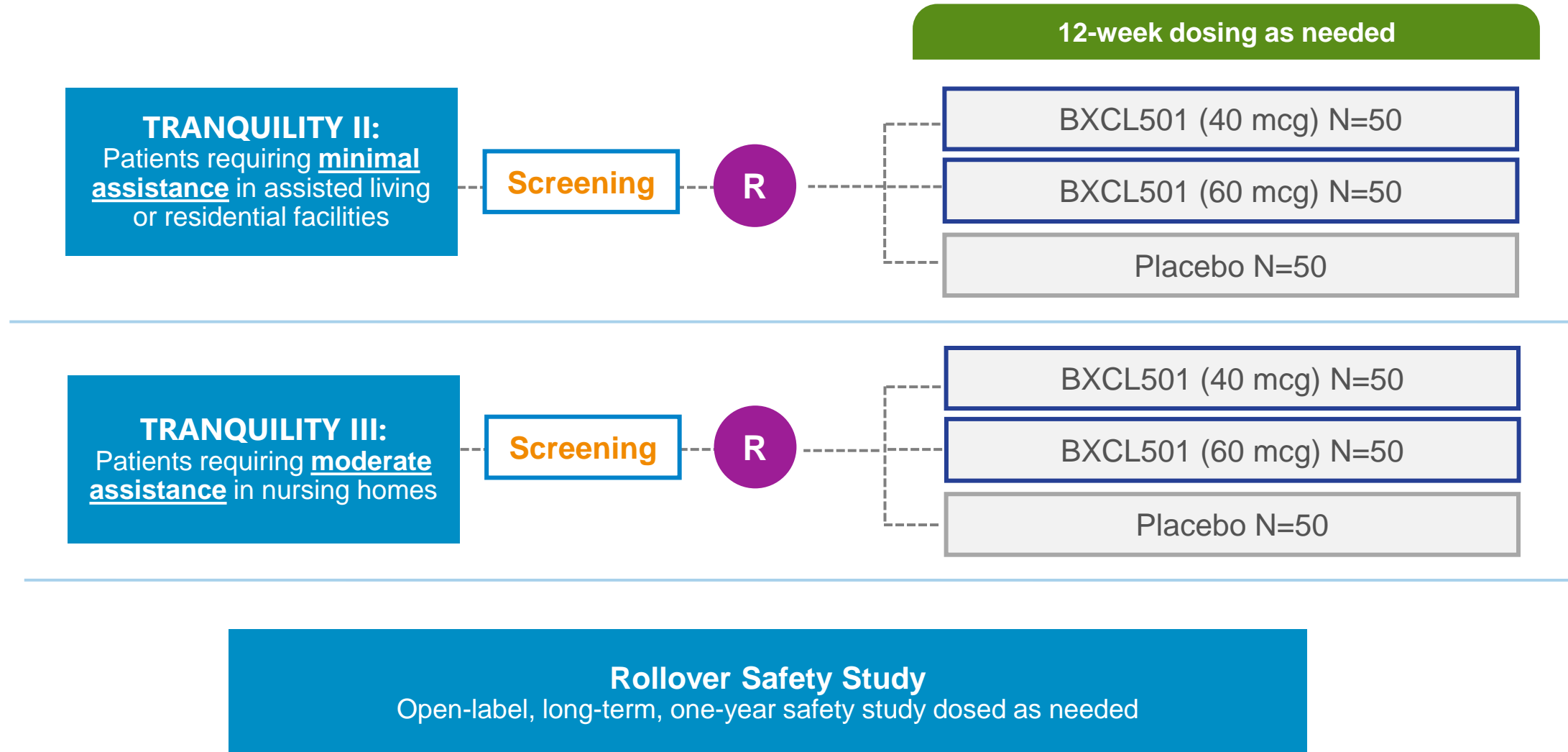
for the acute treatment of agitation associated with dementia

Statistically significant reductions in agitation achieved at 2 hours

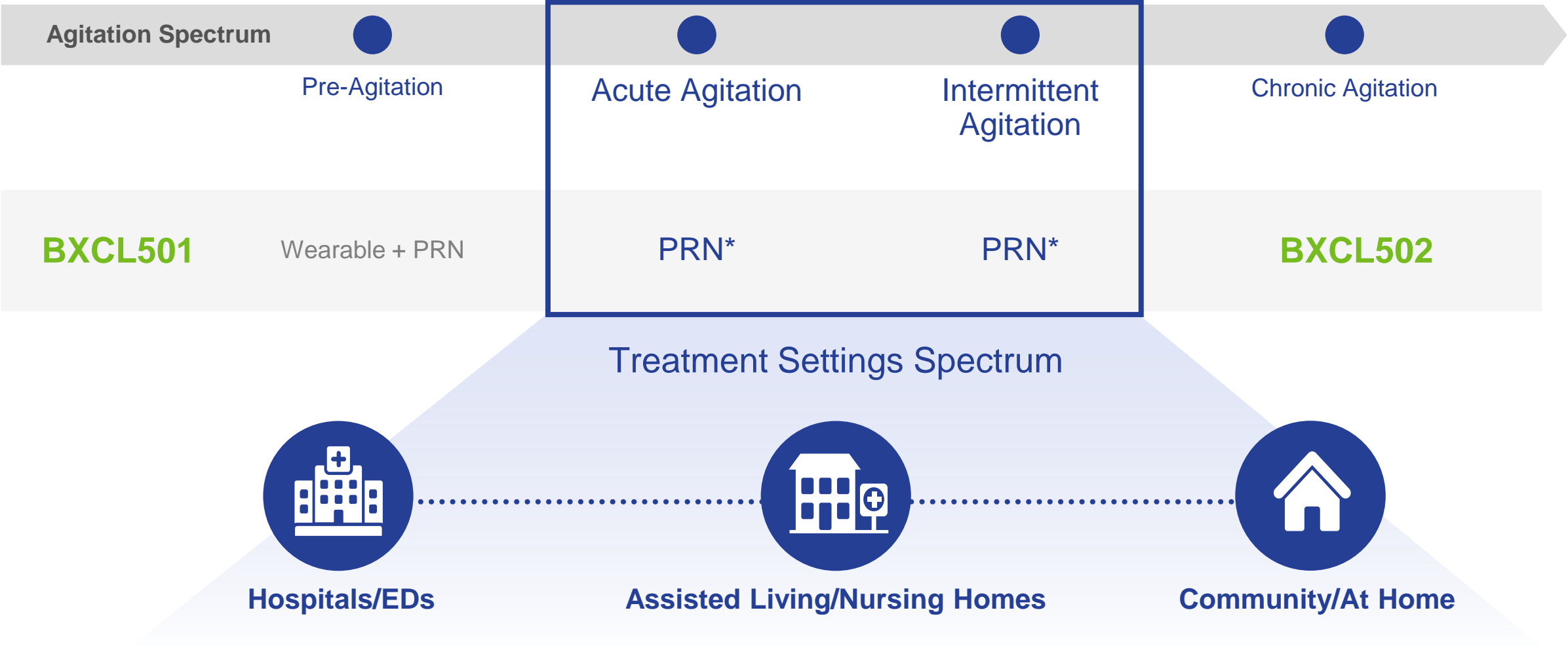


Phase 1b/2 results support initiation of pivotal program for BXCL501 in Alzheimer's disease

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



Alzheimer’s Disease Program Comprehensive Strategy



*As needed

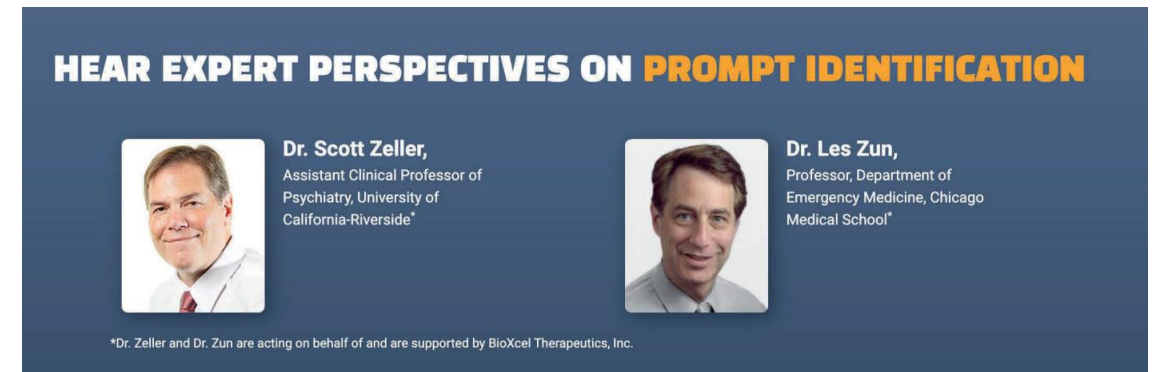
BXCL501

Commercial and Launch Readiness



Informing and Educating Healthcare Professionals

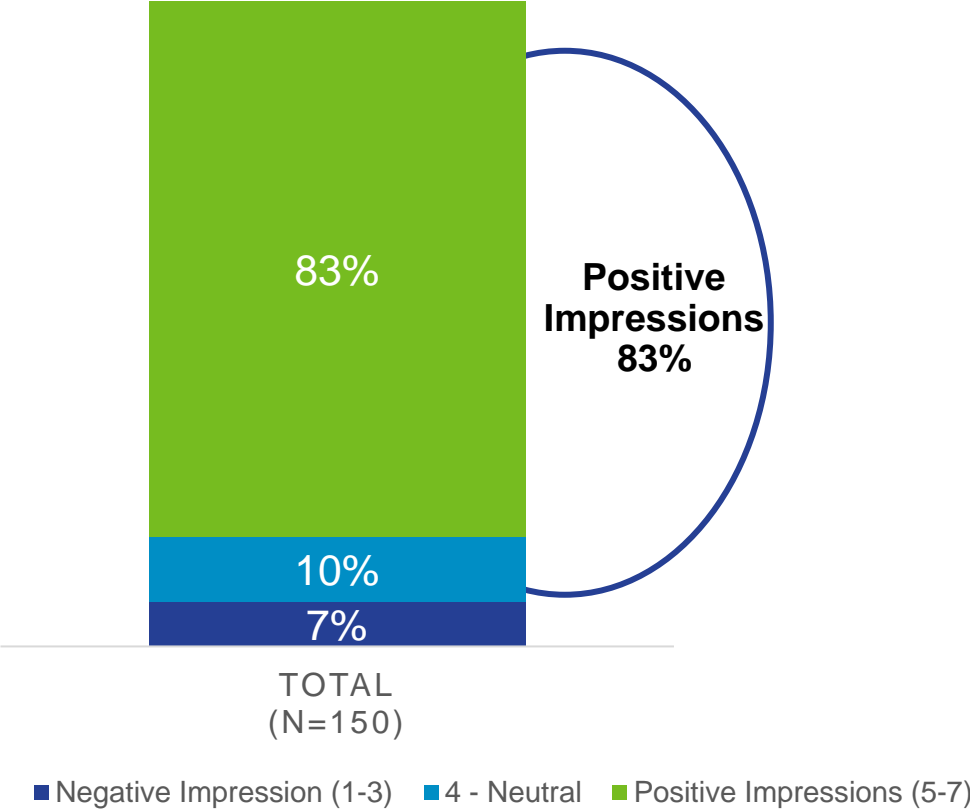
- Fully deployed Medical Science Liaison and Medical Managed Care teams
- Actively engaged with healthcare professionals and payers
- Fully launched unbranded disease education campaign (Including [partnersincalm website](#))
- Participating and presenting at leading conferences
- Submitted pivotal study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



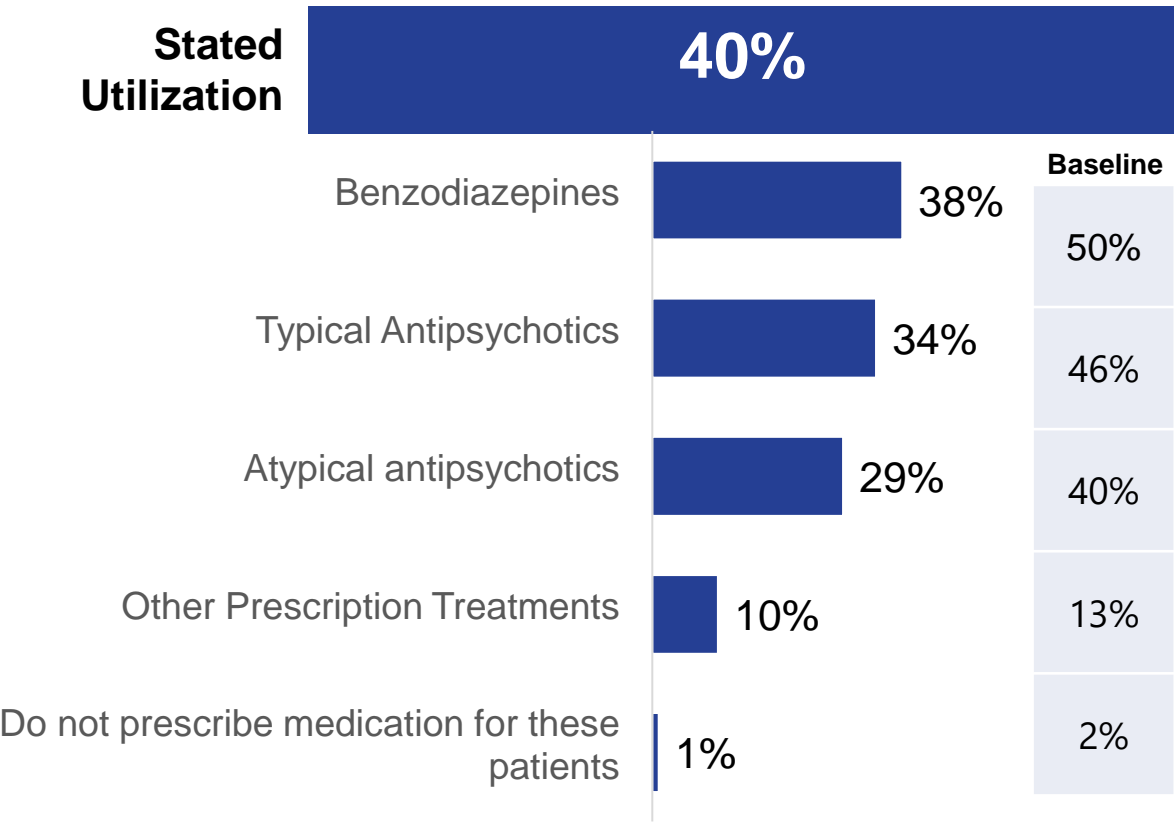
83% of HCPs surveyed have a positive impression of BXCL501*

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

Overall Impression



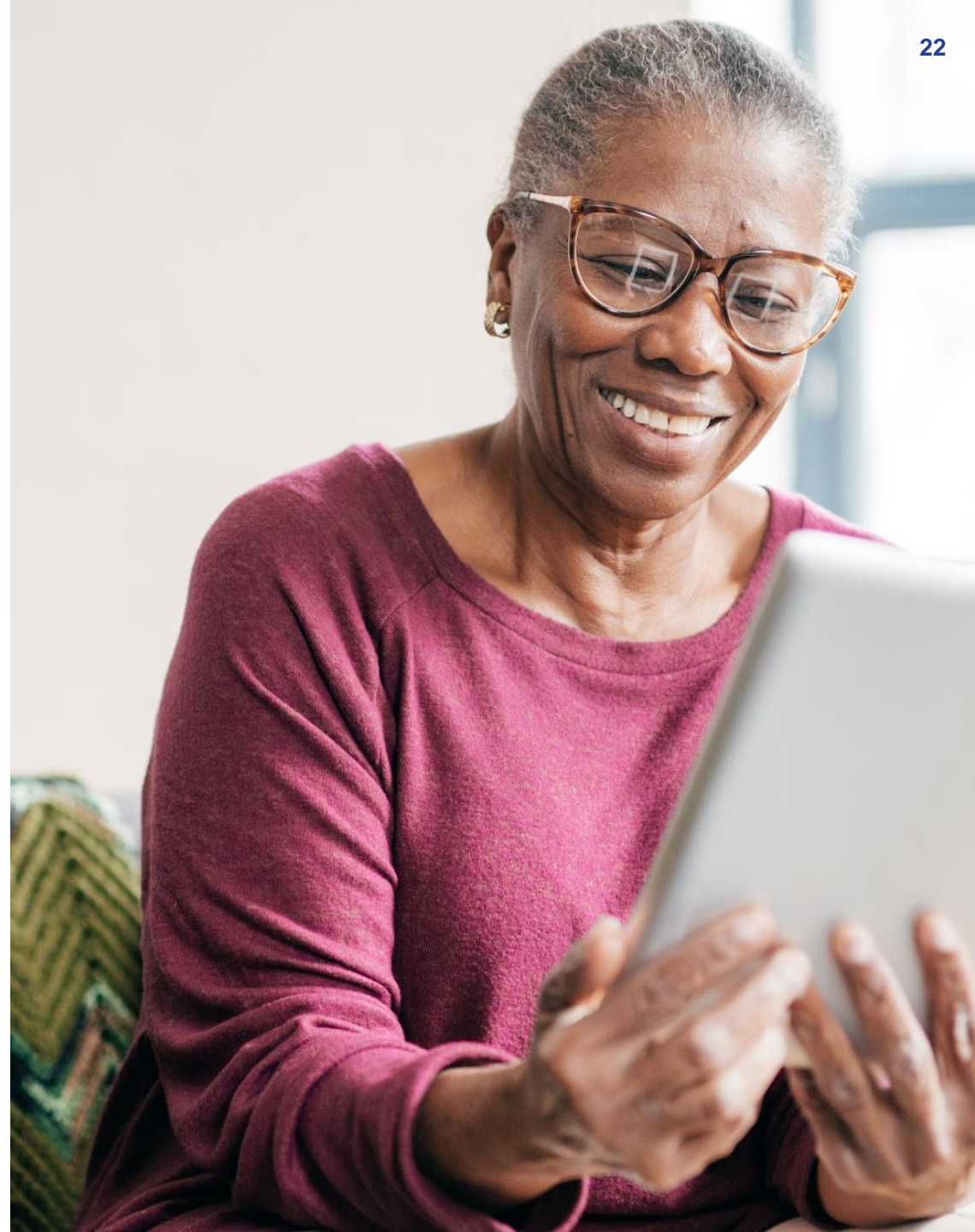
HCP-Stated Utilization



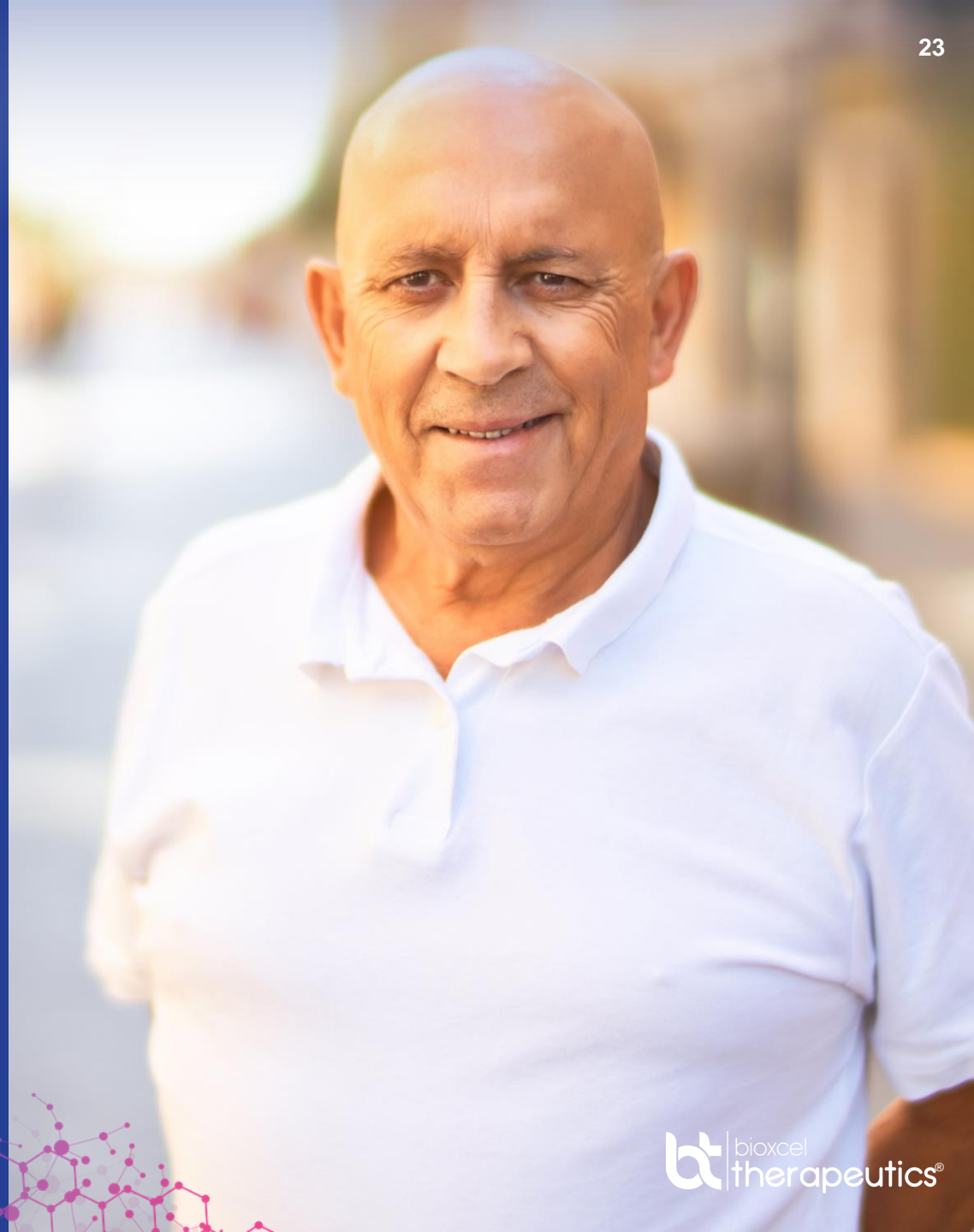
*HCP Quantitative Detail Aid Research (n=150), January 2022

Ready to 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
- Expanded sales leadership and building a national sales team

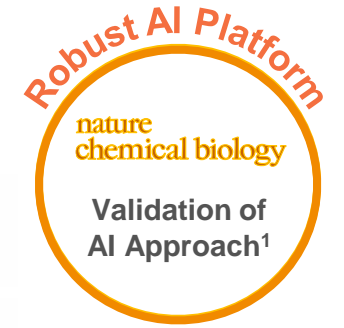


Immuno-oncology Franchise



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

Candidate	<ul style="list-style-type: none"> One of most advanced orally available innate activators in the clinic De-risked: single agent activity + large safety database
Function / MoA Biomarker	<ul style="list-style-type: none"> Designed to: <ul style="list-style-type: none"> Mediate increase in key pro-inflammatory cytokines Activate inflammasome via DPP 8/9 Indications chosen based on frequency of DPP mutations
Clinical Efficacy	<ul style="list-style-type: none"> Pro-inflammatory activity inflames tumor microenvironment and is expected to: <ul style="list-style-type: none"> 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	<ul style="list-style-type: none"> Metastatic castration-resistant prostate cancer — adenocarcinoma and small-cell/neuroendocrine Relapsed Solid Tumors (Hot Tumors)
External Validation	<div>  GILEAD  </div> <div>   </div>



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"

BXCL701 Clinical Development Strategy

Encouraging signals of activity observed in difficult-to-treat tumors in both trials

1

metastatic Castration-Resistant Prostate Cancer — adenocarcinoma and small cell neuroendocrine carcinoma (Cold Tumors):

Phase 1b/2 trial of BXCL701 and KEYTRUDA®

- Safety & initial efficacy data presented at ESMO 2021

2

Relapsed Solid Tumors (Hot Tumors) — 2 cohorts, naïve and resistant to CPIs*:

Open-label Phase 2 basket trial of BXCL701 and KEYTRUDA®; investigator-initiated study led by MD Anderson Cancer Center

- Safety & initial efficacy data presented at ASCO 2021

Efficacy data readout expected in 1Q 2022 (ASCO-GU)



Efficacy data readout expected in 1H 2022

* CPI: Check Point Inhibitors

Clinical Proof of Concept for BXCL701

- Median prior lines systemic therapy = 5
 - All patients pre-treated with ≥ 1 line of TAXANE chemotherapy
- 59% enrolled patients pre-treated with 2 androgen signaling inhibitor
- 31% pre-treated with PROVENGE (sipuleucel-T)

BXCL701 26% composite response rate (n=23)

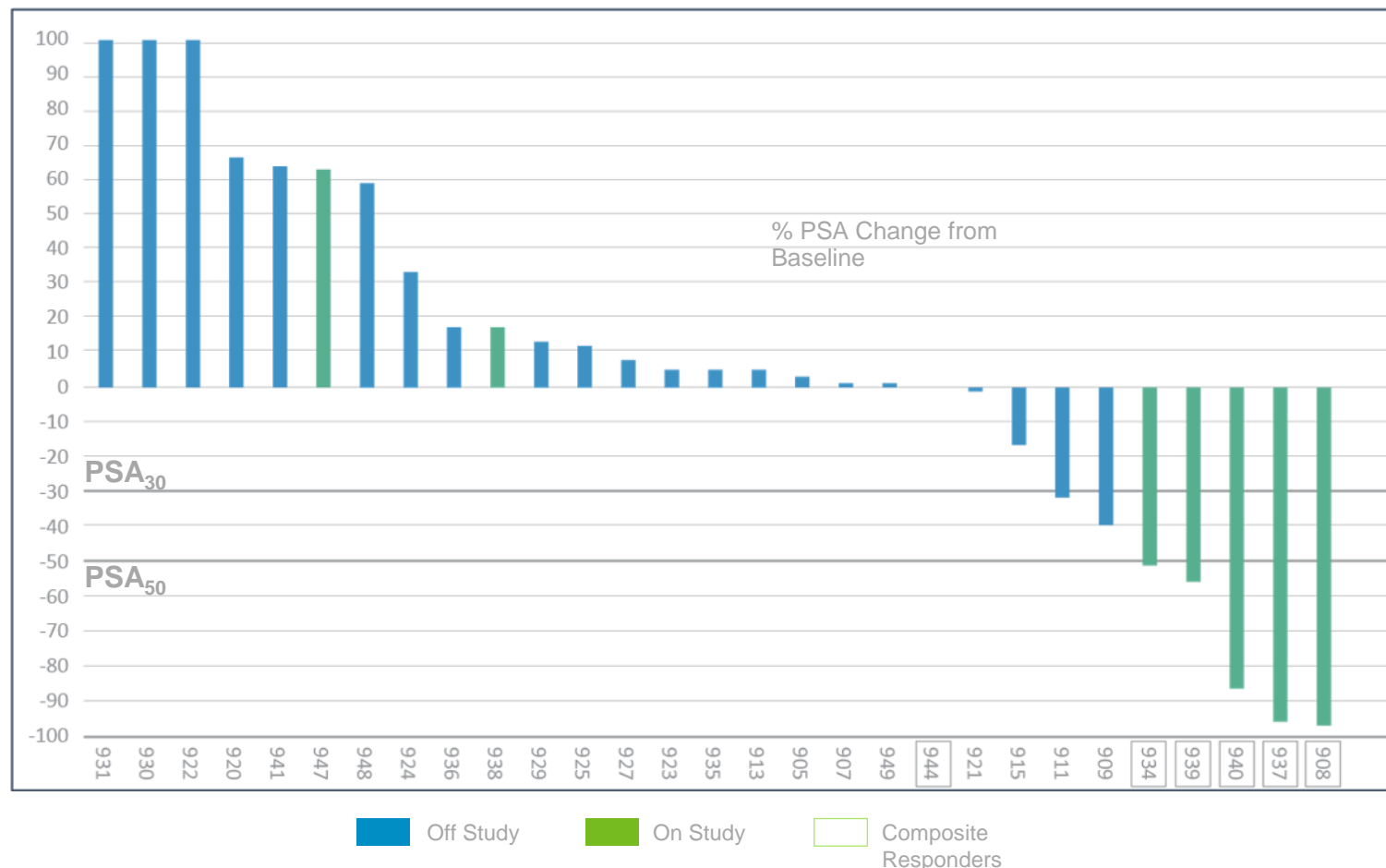
- 16% RECIST-defined PR
- 63% disease control rate (PR + SD + non-CR / non-PD)
- 17% PSA₅₀ including 3 patients with PSA drop $\sim 90\%$

KEYTRUDA single agent historic data^{1*}

- Objective response rate 2-5%
- Disease control rate 12%
- PSA₅₀ 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 adenocarcinoma phenotype

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

- ✓ Preparing to submit IND and initiate clinical trial in 2022



COMMERCIAL LAUNCH READINESS: BXCL501

- ✓ Ready to launch in U.S.



IMMUNO-ONCOLOGY: BXCL701

Aggressive Form of Prostate Cancer (cold tumor)

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

Relapsed Solid Tumors (hot tumors)

- ✓ Interim efficacy data readout expected in 1H 2022

*Preliminary unaudited cash position as of Dec. 31, 2021

**Pipeline as of Dec. 15, 2021

***Acute treatment of agitation in dementia patients with Alzheimer's disease

Thank you!

