NASDAQ: BTAI

bioxcel therapeutics

Developing Transformative Medicines Utilizing AI Approaches

June 2021

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Forward-Looking Statements

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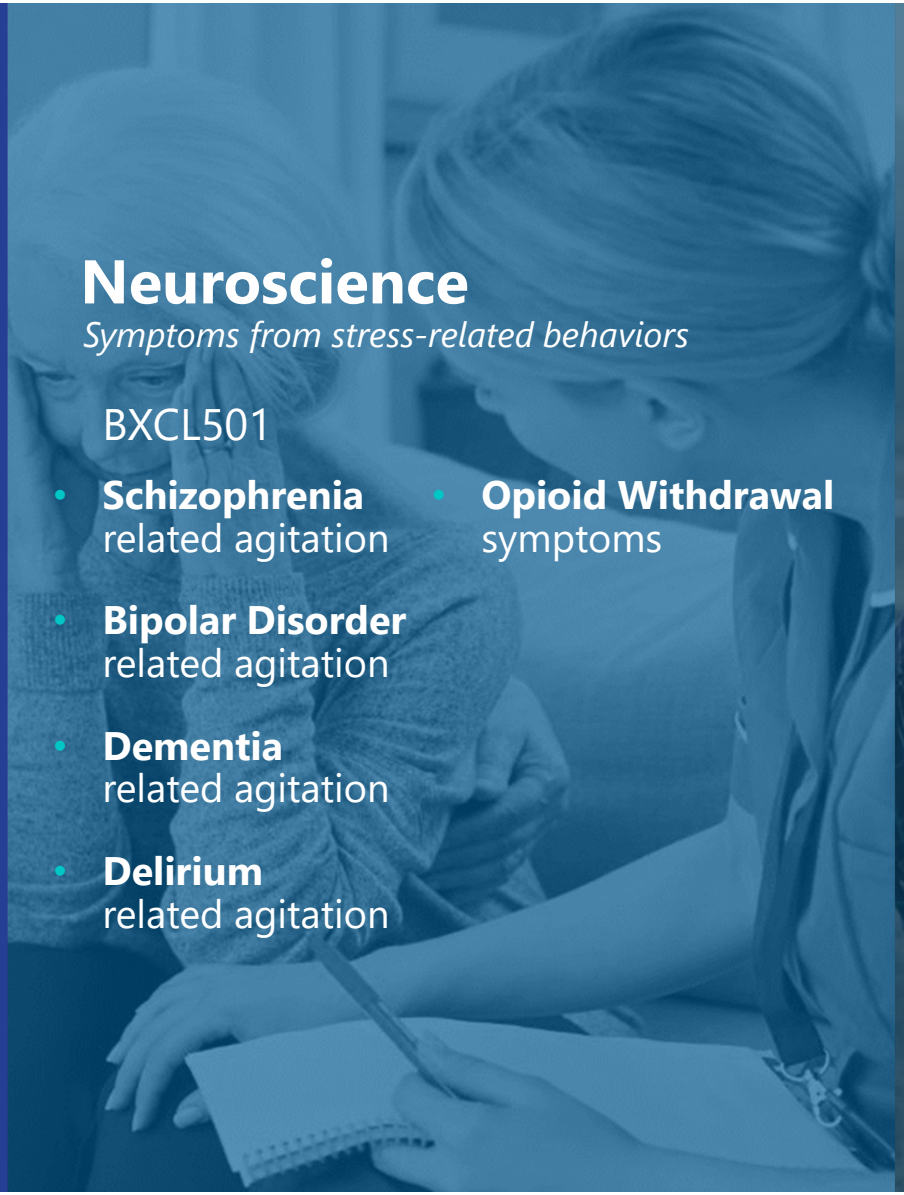
Our mission:

Utilizing AI approaches to develop transformative medicines

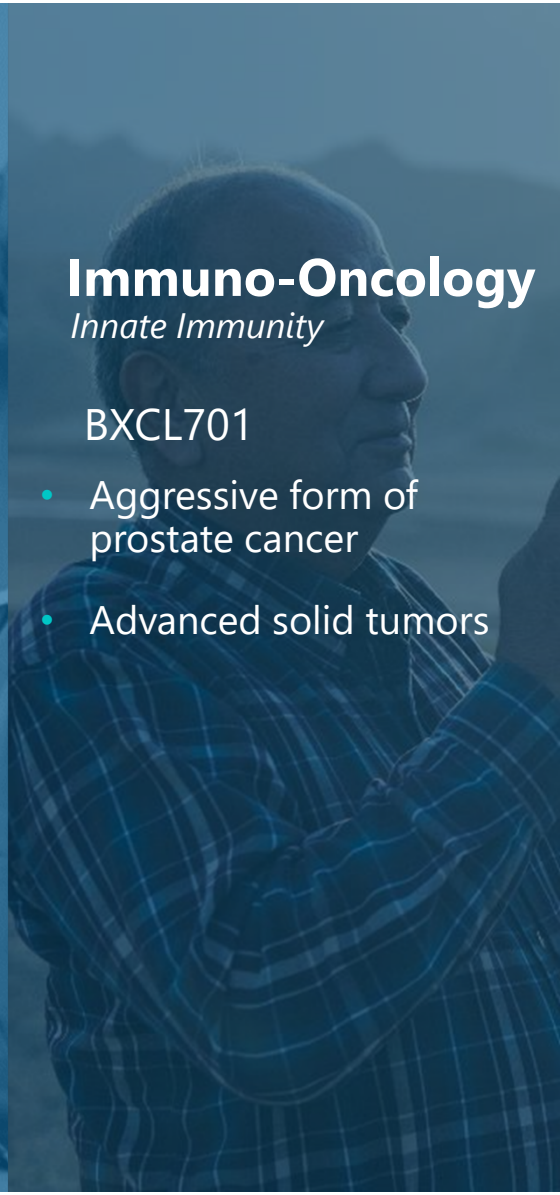


Neuroscience

Symptoms from stress-related behaviors

BXCL501

- **Schizophrenia** related agitation
- **Bipolar Disorder** related agitation
- **Dementia** related agitation
- **Delirium** related agitation
- **Opioid Withdrawal** symptoms



Immuno-Oncology

Innate Immunity

BXCL701

- Aggressive form of prostate cancer
- Advanced solid tumors

AI Platform – Designed to Provide Greater Predictability and Efficiency

BXCL501 – First in-human trial to FDA acceptance for filing of NDA in just over 2 years

Multiple Candidates Screened By AI

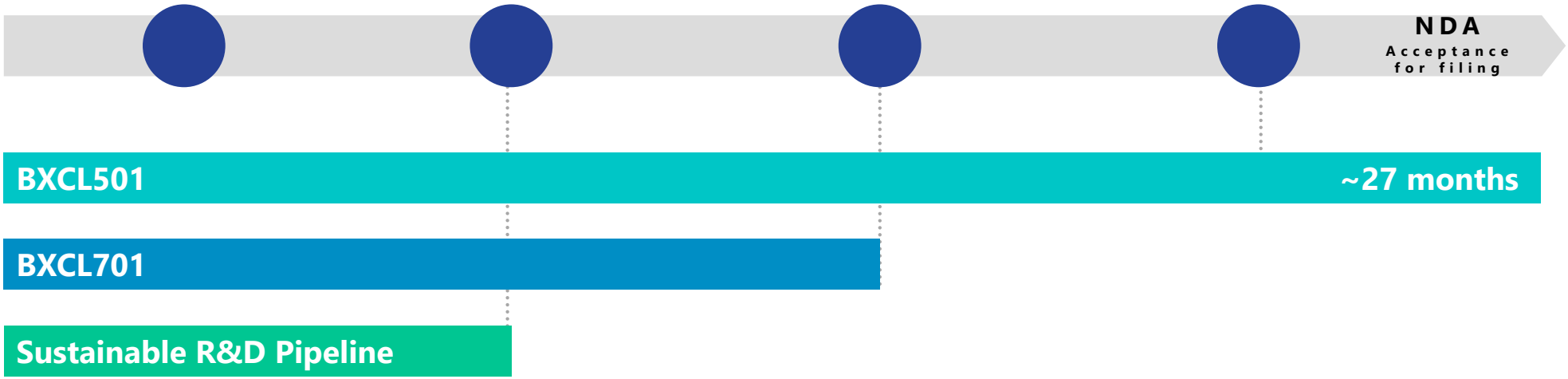
Selection of Best Candidates

Translational Team

Candidate Validation

Clinical Development Team & Regulatory Team

Human Proof of Concept & Registration Trials



Our Pipeline

Neuroscience

BXCL501	
Acute agitation in schizophrenia/bipolar	**SERENITY I & II Trials (PDUFA date – 1/5/22)**
Acute agitation in dementia	**TRANQUILITY Trial (Phase 1b/2 Complete)**
Opioid withdrawal symptoms	**RELEASE Trial (Phase 1b/2 Complete)**
Agitation in delirium*	**PLACIDITY Trial (Phase 2)**
KalmPen™ (Single-use IM)	
Severe acute agitation	**Formulation Development**
BXCL501	
Chronic agitation in dementia	**Clinical Planning**
BXCL501 + combination	
Chronic agitation in dementia	**Formulation Development**
Wearable Device (+BXCL501)**	
Pre & post-agitation in dementia	**Clinical Feasibility Study**

Immuno-oncology

BXCL701	
Castration-resistant prostate cancer (NEPC & adeno)	**Phase 2 (Combination with KEYTRUDA)**
Basket trial – hot tumors (MD Anderson Led)	**Phase 2 (Combination with KEYTRUDA)**

**Delirium study on voluntary pause*

***Regulatory path to be determined; device + drug combination to be evaluated after validation of predictive algorithm*

BXCL501:

Proprietary, Orally Dissolving Thin Film Formulation of Dexmedetomidine (Dex)

Agitation: Debilitating for Patients and Threatening for Healthcare Providers

A Common and Difficult to Manage Symptom

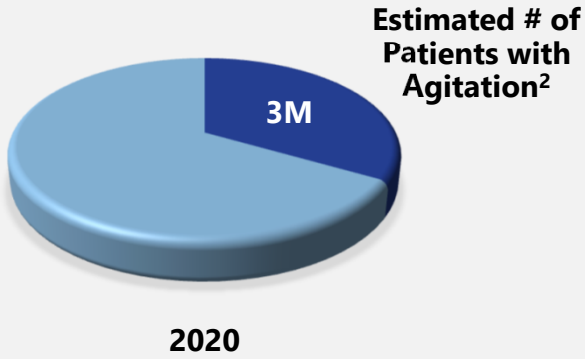
- Agitation is a common occurrence in most neuropsychiatric disorders
- Characterized by recurring episodes requiring frequent treatments
- Over 150M people globally with schizophrenia, bipolar disorder, dementia, delirium and opioid use disorder [1]
    - Over 13M patients in the U.S.[1] experience 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 antipsychotic and benzodiazepines
    - Antipsychotic drugs have black box warnings for elderly
- BXCL501 offers a novel mechanism and a highly differentiated approach

1. Internal company estimates

Significant Commercial Potential in Multiple Indications

Schizophrenia & Bipolar Disorder

Prevalence among Adults ≈ 9M[1]

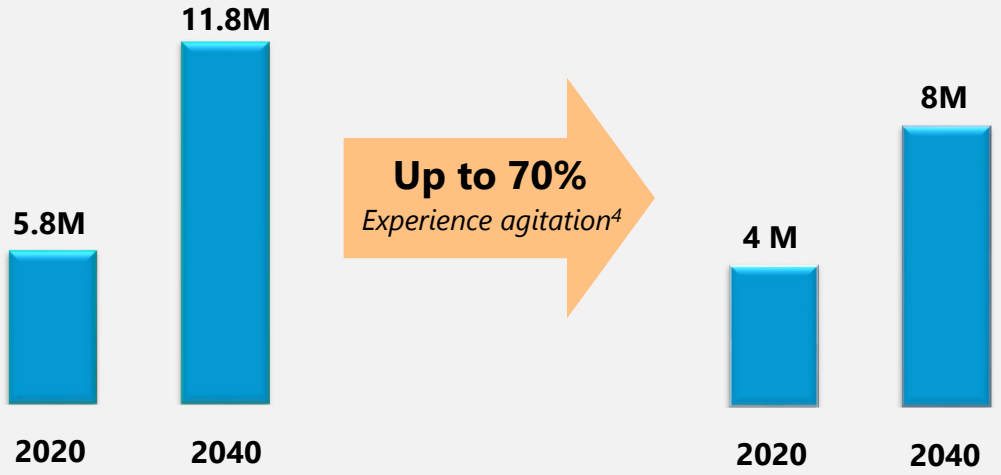


Approximately 40M agitation episodes per year in the U.S. [5]

Dementia

American 65+ with Alzheimer's Disease[2] to double by 2040[3]

Estimated Number of U.S. Patients with Agitation



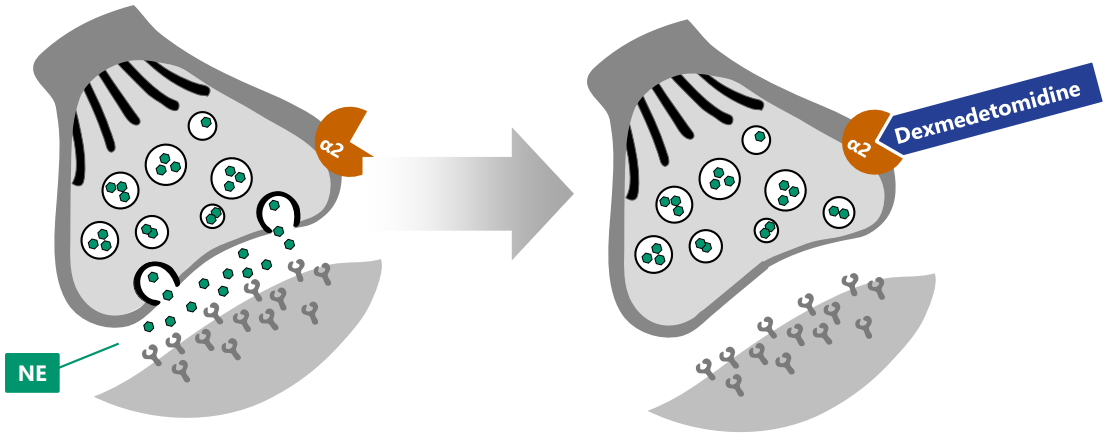
Approximately 100M agitation episodes per year in the U.S. [5]

Sources: 1 Wu, 2006, NAMI; 2 Estimated based on company market research; 3 Alzheimer's Association; 4 Tractenberg, R Neuropsychiatry Clin Neuroscience 14:1 Winter 2002; 5 Estimated based on company market research

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

Patients Successfully Self-Administered Film in Trials

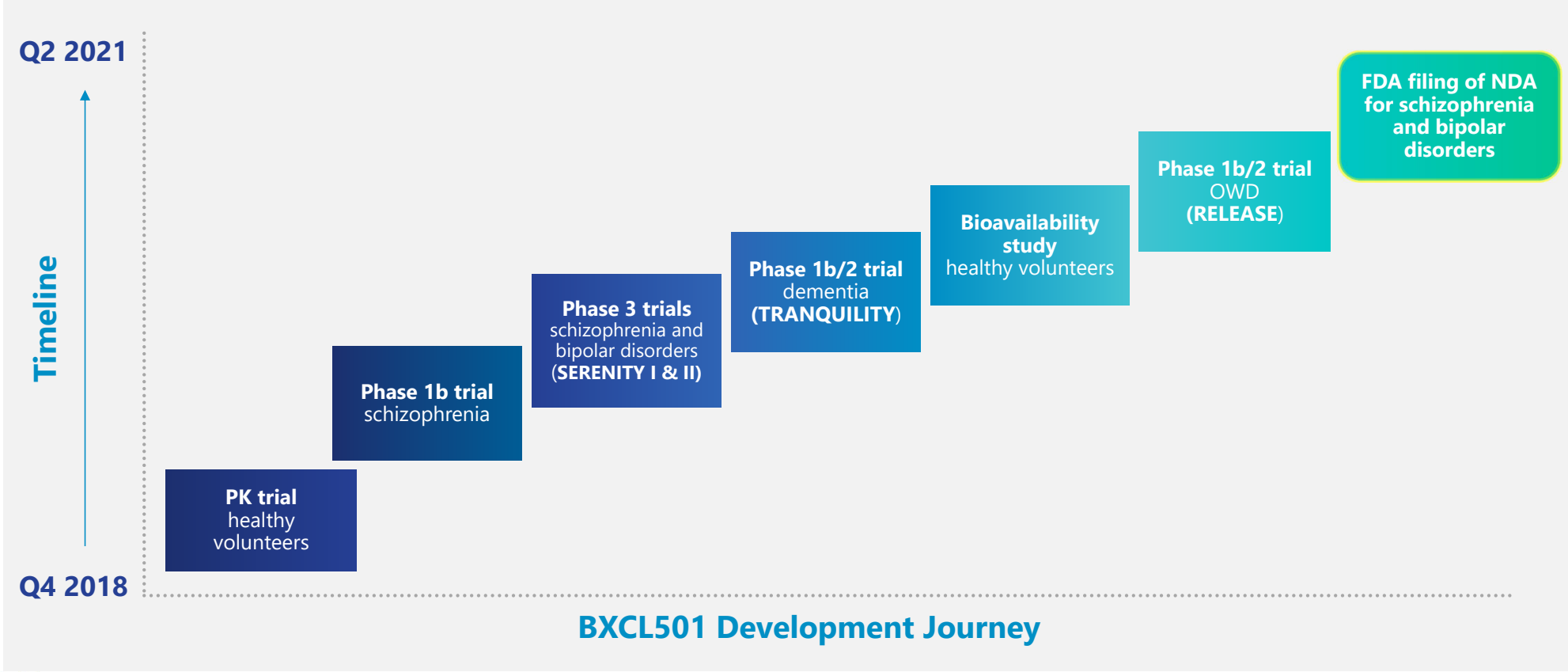
Proprietary, Orally Dissolving, Sublingual or Buccal Thin Film Formulation

- Muco-adhesion properties designed for optimizing compliance
- Designed to be administered behind lower lip or under tongue
- Adaptable technology enables broad dose range
- Flexible for potential combination of multiple drugs on a single film

Transitioned to Commercial Scale-Up Process

Conducted 7 Trials Across a Range of Disorders in >800 Subjects

Was Generally Well Tolerated in Trials Across Three Distinct Indications



SERENITY I & II

Positive Results Effect Observed in Schizophrenia and Bipolar

FDA Fast Track Designation

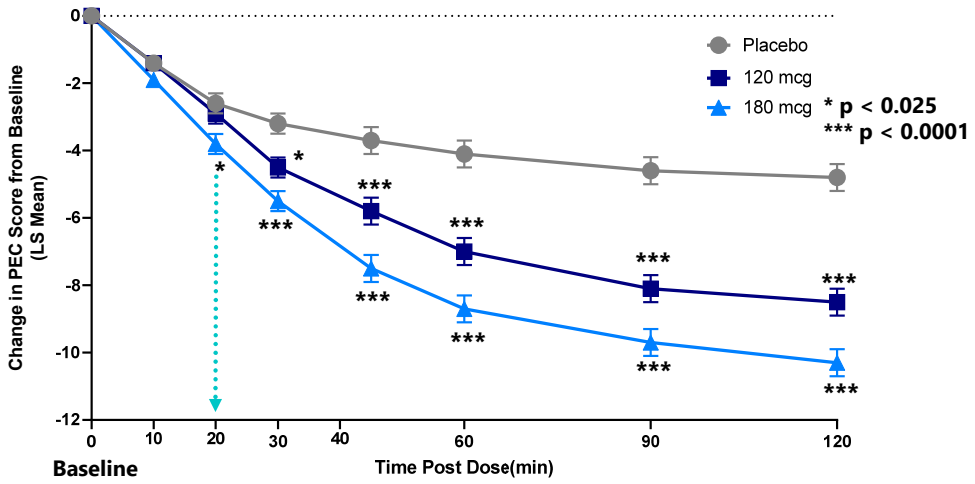
for the acute treatment of agitation associated with schizophrenia and bipolar disorders I & II

- Clinically meaningful, rapid and durable reductions in agitation measures
  - Onset of action in PEC total score observed as early as 20 minutes
  - Response in PEC total score lasted four hours after treatment
- High response rate (~85%) across both populations at 180 mcg dose
- BXCL501 was well tolerated with no severe or serious adverse events

NDA Accepted for filing; PDUFA action date of January 5, 2022

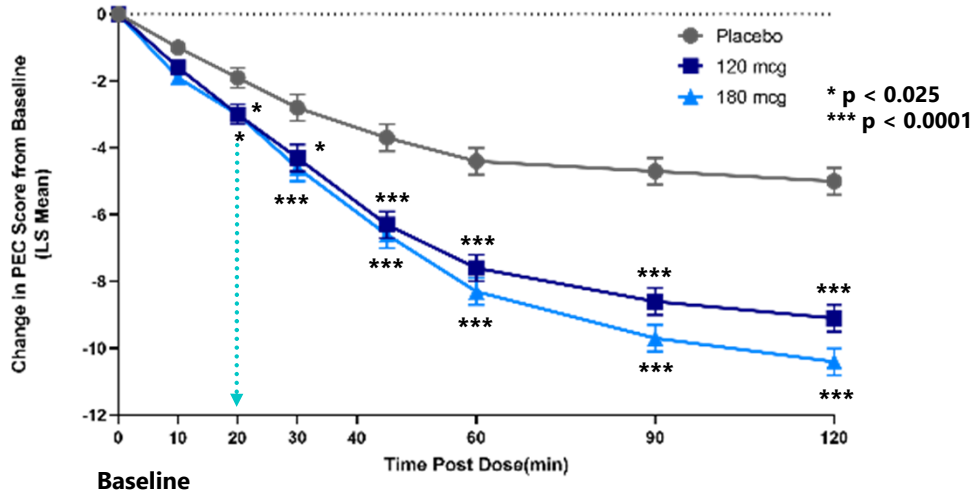
Rapid Onset of Action and Durable Response Observed

SERENITY I: Change in PEC Score from Baseline



Endpoint (120 min)	Placebo	120 mcg	180 mcg
PEC Total score Change from Baseline	-4.8	-8.5 ***	-10.3 ***
Response °	34%	79.1% ***	88.8% ***

SERENITY II: Change in PEC Score from Baseline



Endpoint (120 min)	Placebo	120 mcg	180 mcg
PEC total score change from Baseline	-5.0	-9.1 ***	-10.4 ***
Response °	37%	77.0% ***	90.5% ***

ITT analysis, Least Square Means +/-SEM
° Proportion achieving ≥ 40% PEC reduction

Preparing for Potential Commercialization

Medical Affairs

- ✓ Deployed Medical Scientific Liaisons and Medical Managed Care teams in 1Q 2021
- ✓ Presented several posters at medical meetings highlighting results from SERENITY I and II studies

Commercial

- ✓ Current market research confirms high unmet need and positive reaction to BXCL501 profile
- ✓ Launched "Boiling Point" disease education campaign in alignment with Mental Health Awareness month
- ✓ Payer strategy development underway
  - Key leadership on board
  - Payer and P&T decision maker interactions planned to begin mid-year
- ✓ Sales Force size, structure, and operations infrastructure being finalized

Commercialization Outside U.S.

- Intend to partner in Europe and Japan

TRANQUILITY

Significant Improvement Demonstrated in Dementia Related Agitation

FDA Breakthrough Therapy Designation

for the acute treatment of agitation associated with dementia

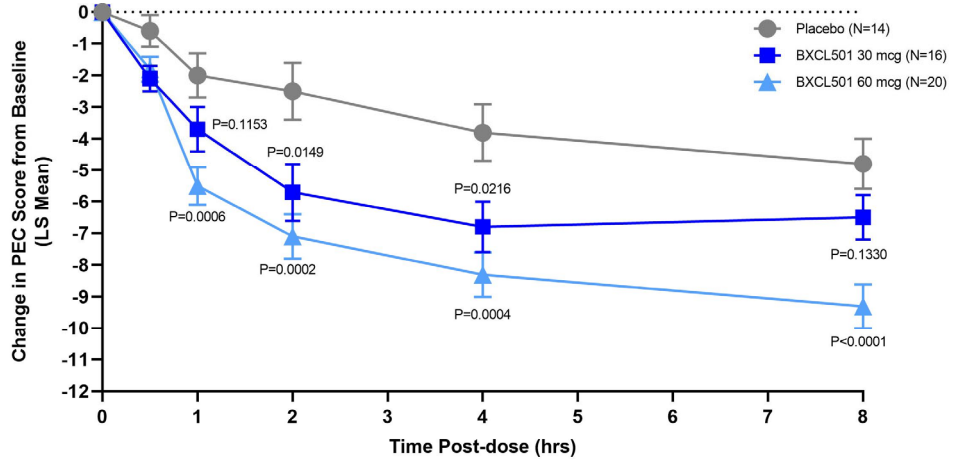
- ✓ Statistically significant reductions in agitation achieved at 2 hours post-dose with both 30 and 60 mcg cohort as measured by the PEC, PAS and Mod-CMAI scales, with:
  - Numerical separation as early as 30 min in PEC score, with significant reductions from baseline observed at 60 min in PEC & PAS scores
  - Duration of response lasted 8 hours after treatment with 60 mcg dose
  - All exploratory endpoints demonstrated significant reductions in agitation with 60 mcg dose
- ✓ BXCL501 was well tolerated with no severe or serious adverse events
- ✓ Higher exposure levels observed in elderly dementia patients potentially enable lower doses

Phase 1b/2 results support initiation of pivotal program for BXCL501 in dementia

TRANQUILITY

Clinically Meaningful, Rapid and Durable Response Observed

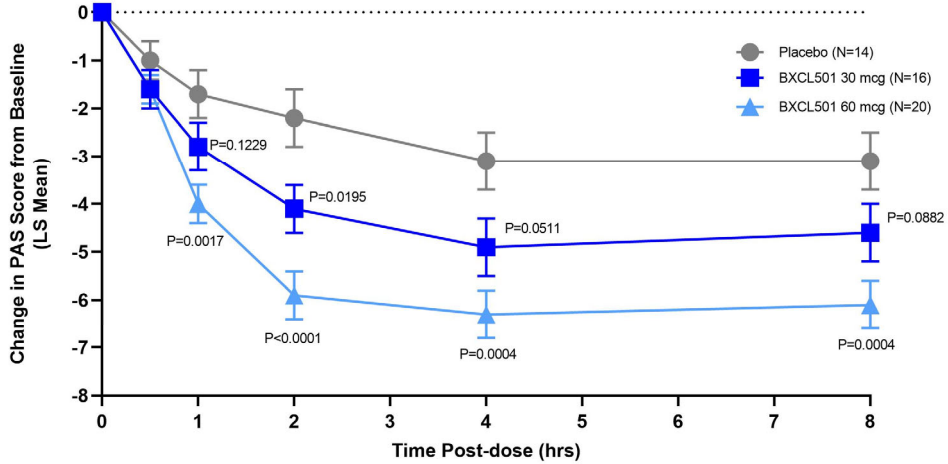
Change in PEC Score from Baseline



P values at 0.5 hrs are 0.0295 for BXCL501 30 mcg and 0.0568 for BXCL501 60 mcg

	Placebo	BXCL501 30 mcg	BXCL501 60 mcg
Change from Baseline at 120 mins (LS Mean)	-2.5	-5.7	-7.1
Response °	0%	31%	70%

Change in PAS Score from Baseline



P values at 0.5 hrs are 0.3162 for BXCL501 30 mcg and 0.2631 for BXCL501 60 mcg

	Placebo	BXCL501 30 mcg	BXCL501 60 mcg
Change from Baseline at 120 mins (LS Mean)	-2.2	-4.1	-5.9

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
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

TRANQUILITY

Dementia Program Comprehensive Strategy

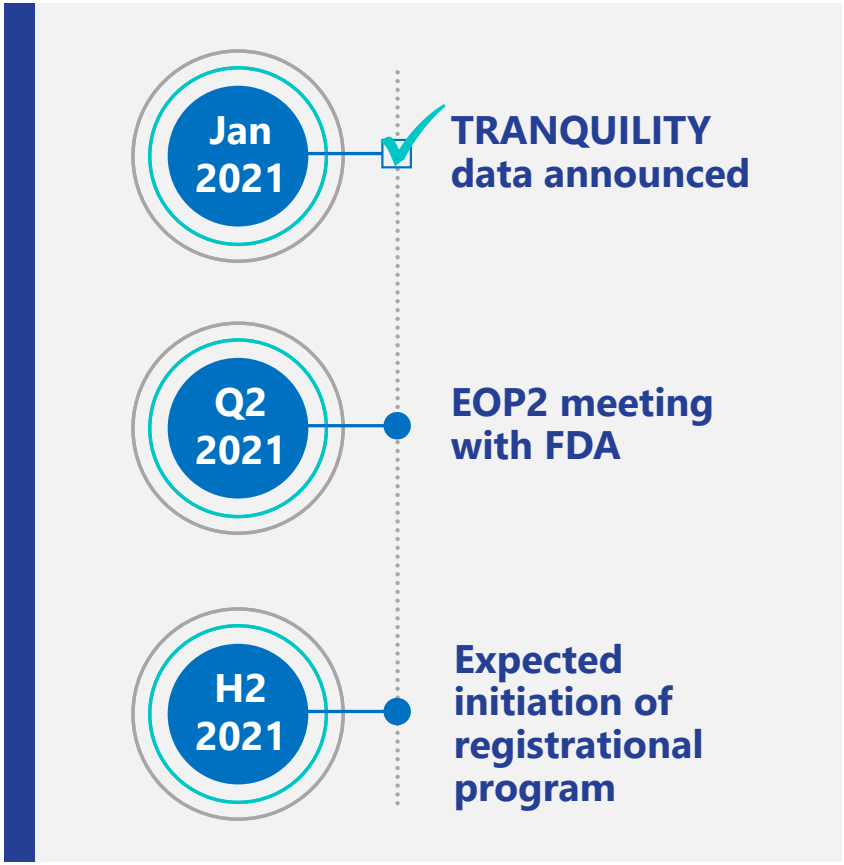
Agitation Spectrum	Pre-Agitation	Acute/Episodic Agitation	Intermittent Chronic Agitation	Chronic Agitation
BXCL501	Wearable + PRN	**PRN***	**PRN***	Daily (BXCL501 single or combination use)

*As needed

TRANQUILITY

Plans for Registrational Studies in Dementia

- End of Phase 2 meeting with FDA held in 2Q 2021 with additional meetings planned in 2H 2021 to discuss Phase 3 program
- Expected initiation of Phase 3 studies in 2H 2021

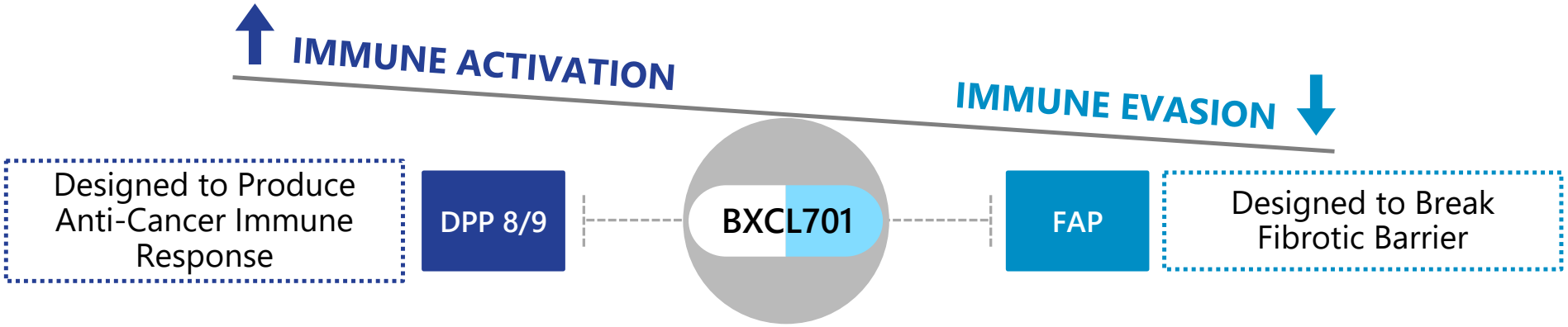


BXCL701

Investigational Oral IO Therapy

Orally Administered Investigational Activator of Systemic Innate Immunity Pathway

Dual MoA designed to inhibit DPP 8/9 & FAP



- BXCL701 is designed to stimulate the innate immune system, facilitating a strong adaptive anti cancer immune response to:
  - Expand activity of immune agents into cold tumors
  - Reverse resistance in checkpoint-treated hot tumors
  - Augment responses in checkpoint naïve hot tumors

BXCL701 Clinical Development Strategy

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

1

Castrate Resistant Prostate Cancer—Adeno and tNEPC (Cold Tumors):

Phase 1b/2 trial of BXCL701 and KEYTRUDA

- Safety & initial efficacy data presented at SITC

2

Solid Tumors Responsive (Hot Tumors) to CPIs*- naïve and resistant cohorts:

Open-label Phase 2 basket trial led by MD Anderson

- Safety & initial efficacy data presented at SITC

Efficacy data readout expected in 2H 2021

Plans to present results at major medical conferences

* CPI: Check Point Inhibitors

What's Ahead

Key Clinical and Commercial Catalysts for 2021

Strong cash position $194M to fund key milestones*

NEUROSCIENCE – BXCL501

Schizophrenia & Bipolar:

- ✓ NDA submitted in March 2021
- ✓ NDA accepted for filing – PDUFA date of 1/5/2022
- • MAA submission to EMA anticipated in 2H 2021

Dementia:

- ✓ Reported positive topline results from the TRANQUILITY Phase 1b/2 trial
- ✓ TRANQUILITY 40 mcg supplemental cohort study underway
- • EOP2 meeting with the FDA held in 2Q 2021; additional meetings planned in 2H 2021 to discuss Phase 3 program
- • Expected initiation of Phase 3 studies in 2H 2021

Opioid Withdrawal Symptoms:

- ✓ Reported topline results for RELEASE Phase 1b/2 trial

COMMERCIAL STRATEGY

Commercial Readiness Preparations

- ✓ Payer strategy development underway
- ✓ Launched disease education campaign
- • Sales Force size, structure, and operations infrastructure being finalized
- • Sales Force on board in 4Q 2021

IMMUNO-ONCOLOGY – BXCL701

Aggressive Form of Prostate Cancer (cold tumor):

- ✓ Met prespecified efficacy bar in ongoing adenocarcinoma Phase 1b/2 trial
- • More complete efficacy data expected to be presented at a medical conference later this year

Solid Hot Tumors – Basket Trial:

- • Efficacy data readout expected 2H 2021

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*Reported cash position as of March 31, 2021

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Thank You!

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