NASDAQ: BTAI

bioxcel therapeutics

39th ANNUAL J.P. MORGAN HEALTHCARE CONFERENCE

January 14, 2021

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, statements that relate to the advancement and development of BXCL501 and BXCL701, anticipated milestones, clinical development plans, including registrational studies for BXCL501 in dementia patients, the availability and results of data from clinical trials, planned commercialization expected market size 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's current expectations and various assumptions. BioXcel believes there is a reasonable basis for its expectations and beliefs, but they are inherently uncertain.

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Our mission:

Utilizing AI approaches to develop transformative medicines

Neuroscience

Symptoms from stress-related behaviors

- **Schizophrenia** related agitation
- **Bipolar Disorder** related agitation
- **Dementia** related agitation
- **Delirium** related Agitation
- **Opioid withdrawal** symptoms

Immuno-Oncology

Innate Immunity

- Aggressive form of prostate cancer
- Advanced solid tumors

AI Platform – Greater Predictability and Efficiency

BXCL501 – First-in-Human to Pivotal Data in 20 Months

Multiple Candidates Screened By AI

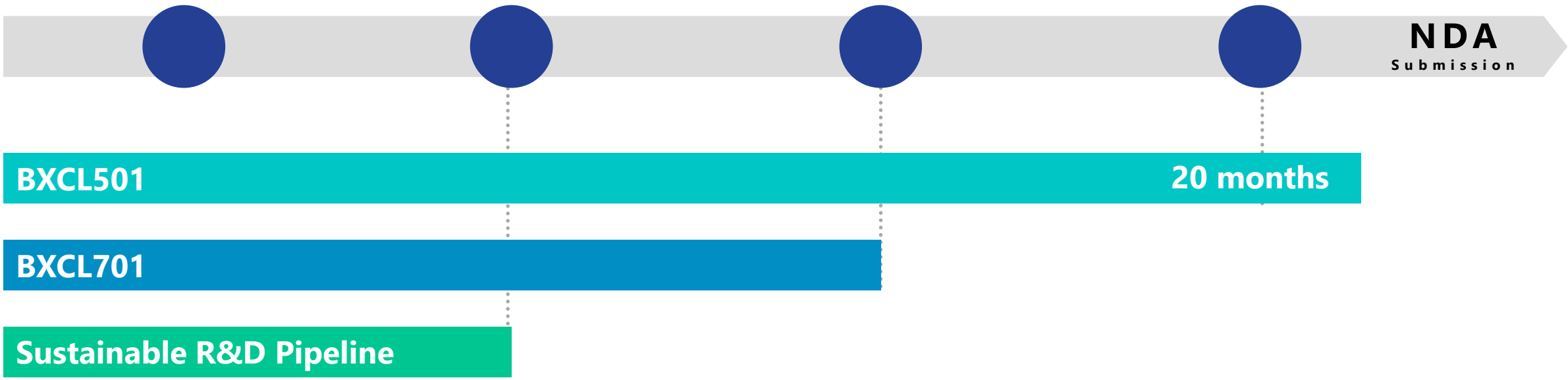
Selection of Best Candidates

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 (Phase 3 Complete – NDA Planned for Q1 2021)**
Acute agitation in dementia	**TRANQUILITY Trial (Phase 1b/2 Complete)**
Opioid withdrawal symptoms	**RELEASE Trial (Phase 1b/2)**
Agitation in delirium	**Phase 2 initiation planned**
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)**

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

Neuroscience Franchise Expansion Plan

Total Disease Prevalence Across Multiple Indications >50M patients in U.S.[1]

Anticipated Launch Timing

Bipolar Disorders

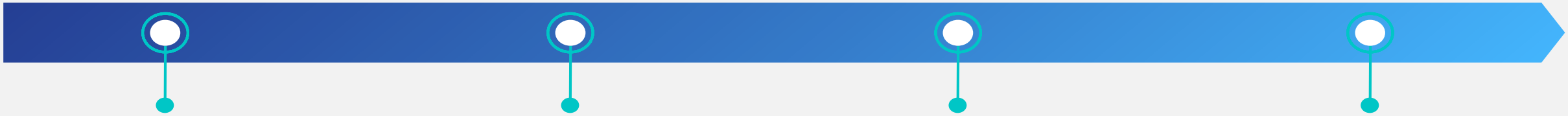
Schizophrenia

Dementia

Opioid Withdrawal Symptoms

Delirium

Other Potential Indications:
- **Depression Related Agitation**
- **Anxiety Disorders**
- **PTSD**
- **Traumatic Brain Injury**



>200M Agitation Episodes Per Year in the U.S.[1]

Value Drivers

- Treatment settings: institutional to community
- Broad spectrum of agitation: acute to chronic

1. Internal company estimates

Commercialization Strategy for Schizophrenia & Bipolar Related Agitation

Strong Commercial and Medical Leadership

Will Kane
Chief Commercial Officer

Allergan · Pfizer · SEPRACOR

Reina Benabou, M.D., Ph.D.
Chief Development Officer

cognivue · Pfizer · NOVARTIS

Building Cross-Functional Team

- Medical: MSL team in training for expected deployment in March 2021
- Market Access: Payer and P&T Decision Maker Interactions planned to begin mid-year
- Sales: 75-100 representative sales force anticipated to be on board in Q4 2021

Commercialization Outside U.S.

- Intend to partner in Europe and Japan

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

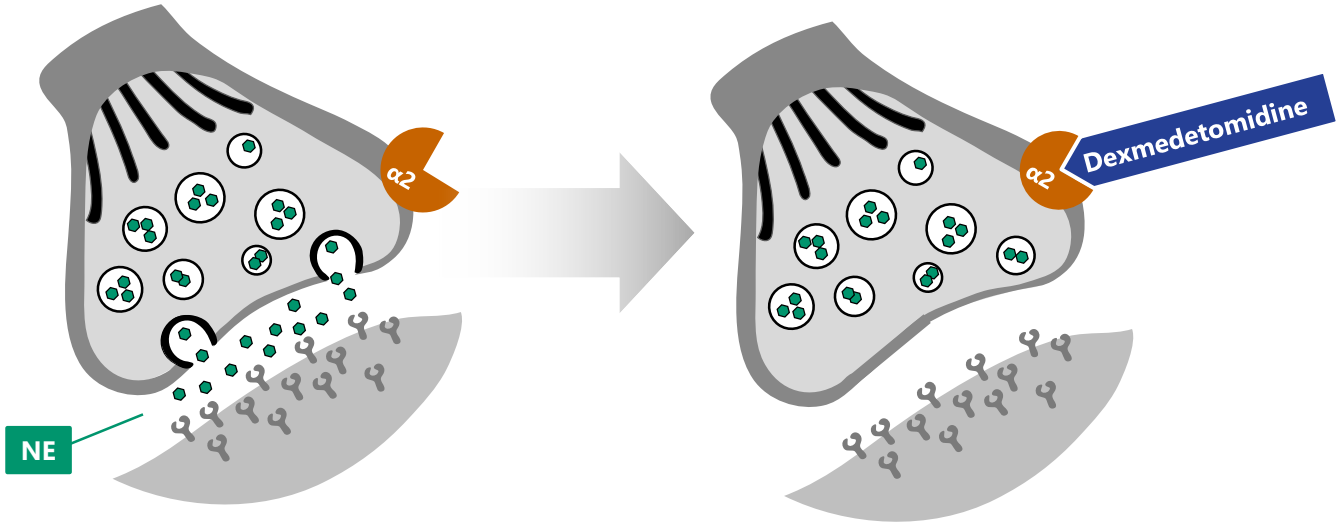
- Agitation is a common occurrence in most neuropsychiatric disorders
- Characterized by recurring episodes requiring frequent treatments
- Over 150M people globally[1] with schizophrenia, bipolar disorder, dementia, delirium and opioid use disorder
  - Over 13M patients in the U.S.[1] experience agitation 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

BXCL501: Novel Mechanism Potentially Targets Causal Agitation

Positive Trials in Three Distinct Indications Support Underlying MoA

Dexmedetomidine MoA



(+) Agitation **(-) Agitation**

Norepinephrine (NE)

*Dexmedetomidine

Highly Differentiated from Current Treatments

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

Patent Portfolio

- ✓ U.S. patent (No. 10,792,246) issued
- ✓ IP protection expected until 2039
- ✓ Multiple patent applications

Robust Treatment Effect Observed in Schizophrenia and Bipolar

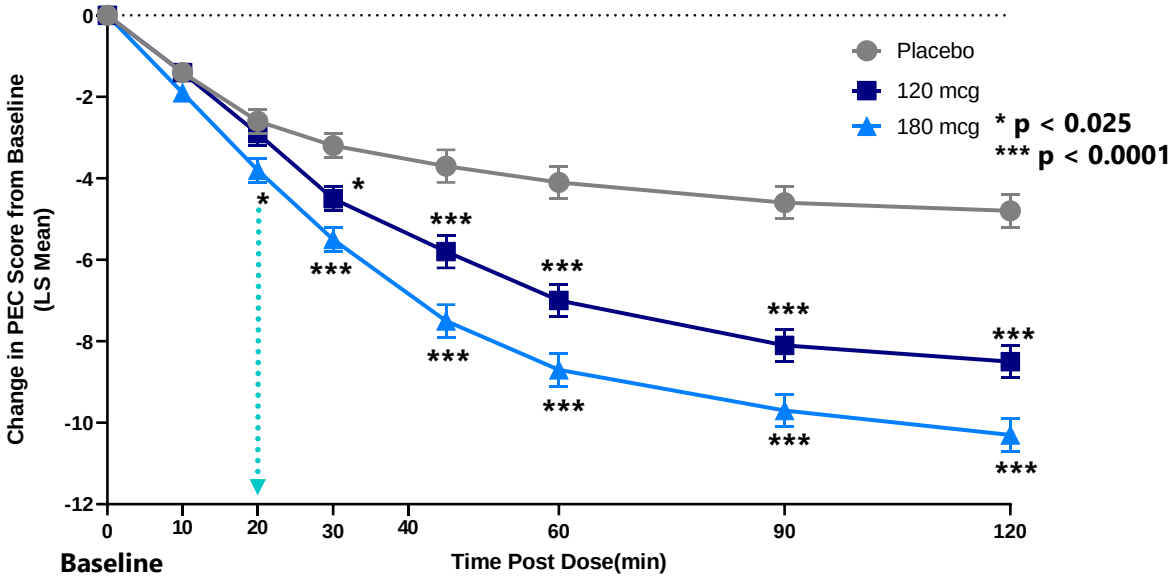
Fast Track Designation

- ✓ Clinically meaningful, rapid and durable reductions in agitation achieved
  - Onset of action in PEC score observed as early as 20 minutes
  - Durable response lasting at least four hours after treatment
- ✓ High response rate (~85%) across both populations
- ✓ BXCL501 was well tolerated with no severe or serious adverse events

Complete NDA submission to U.S. FDA planned for Q1 2021

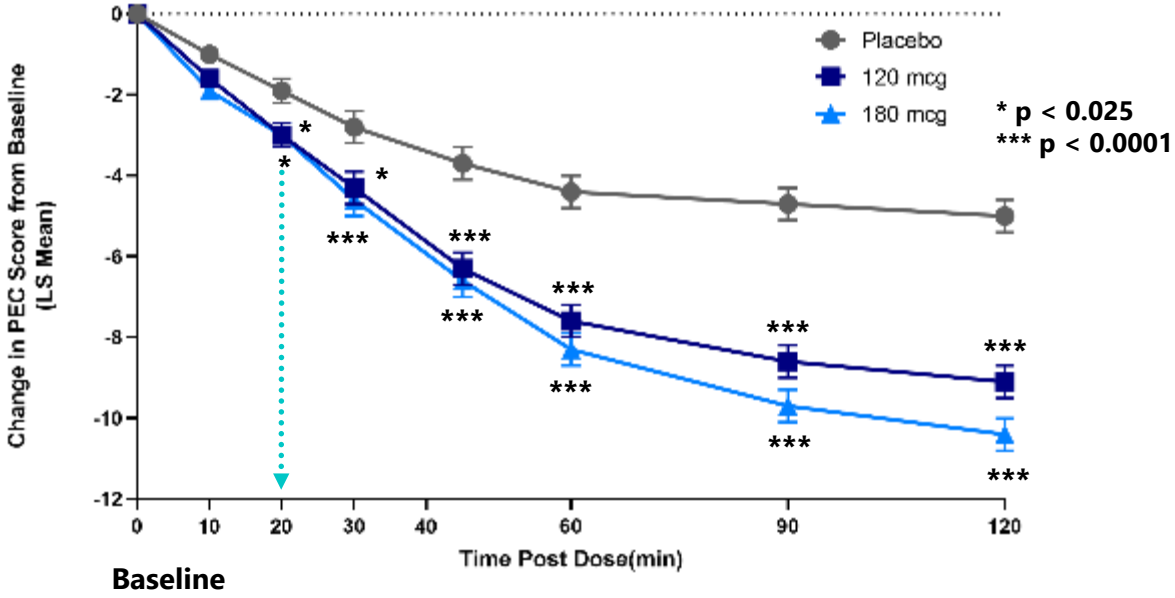
Rapid Onset of Action and Durable Response Observed

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%	67% ***	87% ***

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%	69% ***	85% ***

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

Significant Improvement Demonstrated in Dementia Related Agitation

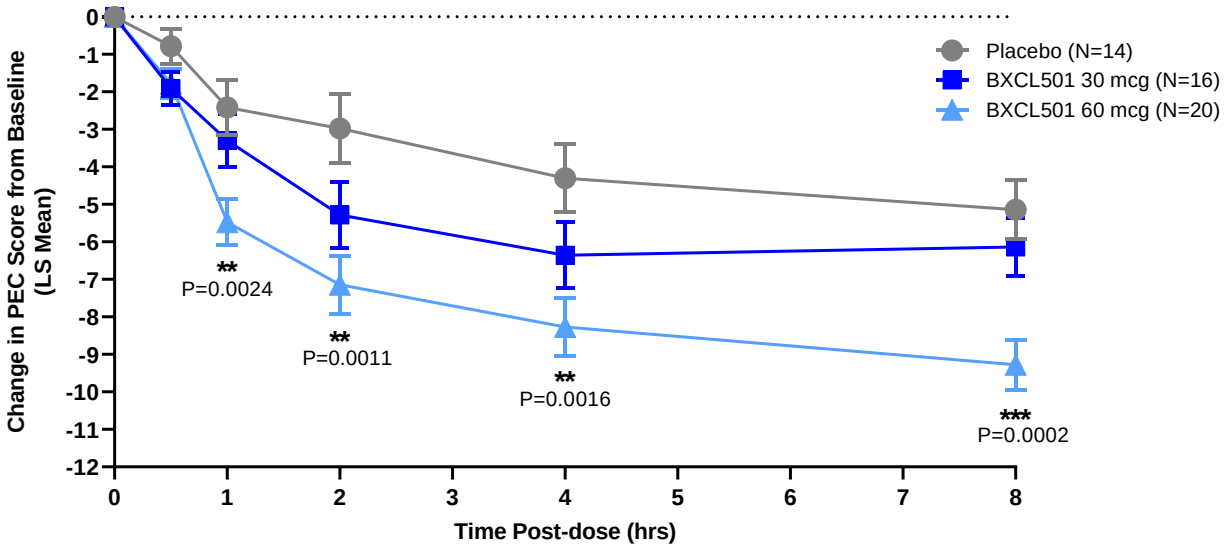
Fast Track Designation

- ✓ Clinically meaningful, rapid and durable reductions in agitation achieved with 60 mcg cohort as measured by all scales:
  - Numerical separation as early as 30 minutes, with statistically significant reductions at 60 minutes
  - Reduction in PEC and PAS scores lasted 8 hours after treatment
- ✓ 30 mcg dose cohort showed numerical improvements across all scales
- ✓ BXCL501 was well tolerated with no severe or serious adverse events
- ✓ Higher exposure levels observed in elderly dementia patients potentially enable efficacy at lower doses

Results provide a clear path to pivotal program for BXCL501 in dementia

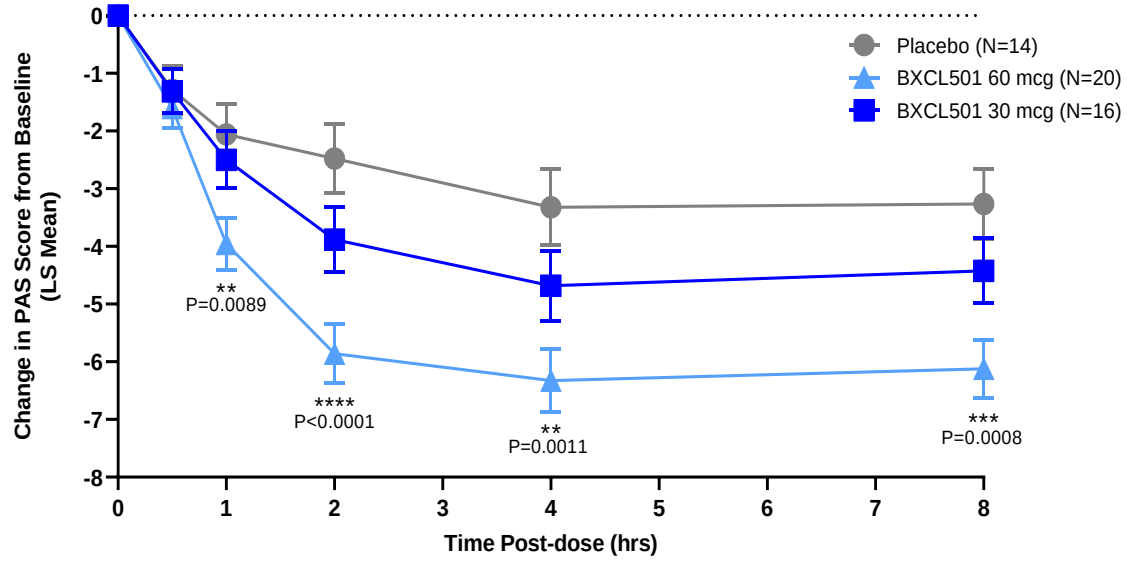
Clinically Meaningful, Rapid and Durable Response

Change in PEC Score from Baseline



	Placebo	BXCL501 30 mcg	BXCL501 60 mcg
Change from Baseline at 120 mins (LS Mean)	-2.9	-5.4	-7.1 **
Response °	7%	25%	70% **

Change in PAS Score from Baseline



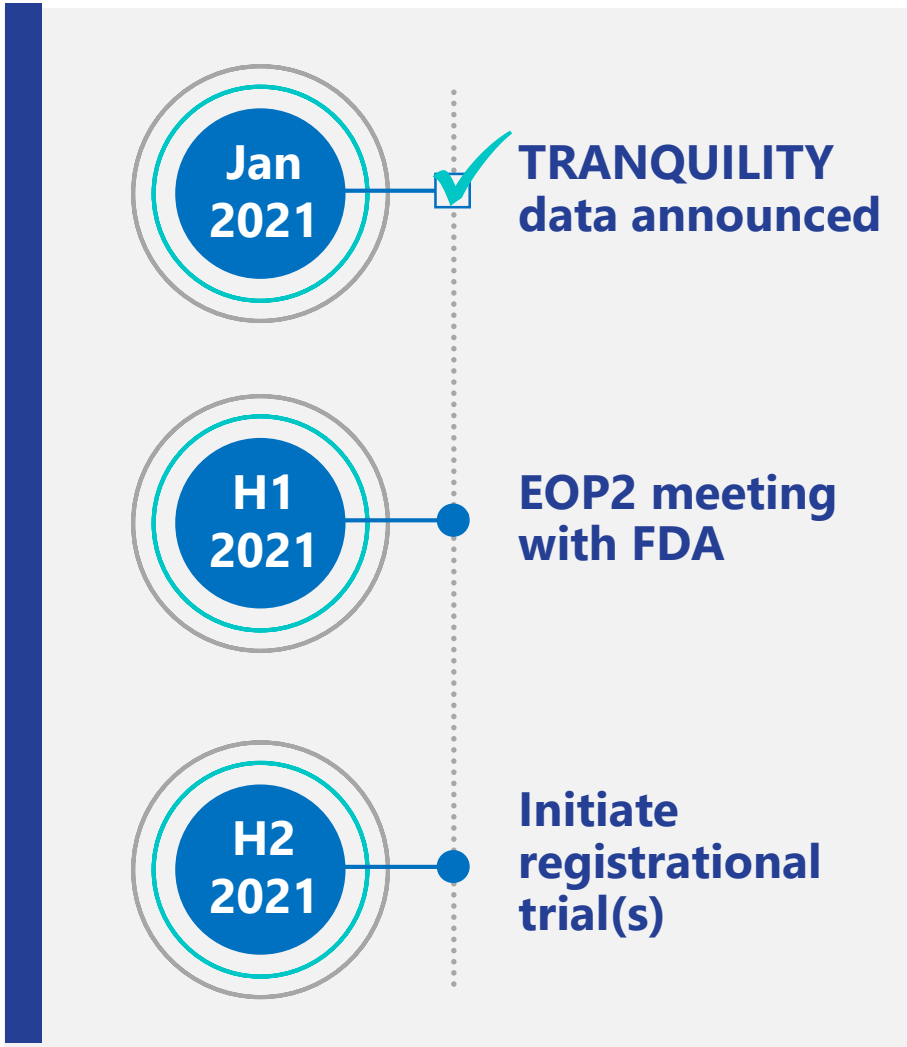
	Placebo	BXCL501 30 mcg	BXCL501 60 mcg
Change from Baseline at 120 mins (LS Mean)	-2.5	-3.9	-5.9 ****

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

Plans for Registrational Studies in Dementia

- End of Phase 2 meeting with FDA planned for H1 2021
- Potential elements of a registrational program (Phase 3):
  - Study(s) expected to include 300-400 patients
  - Two doses to be evaluated
  - Endpoints may consist of: PEC, PAS, Mod-CMAI, CGI-I or ACES
  - Clinical sites to include assisted living centers, nursing homes and hospitals
- Aiming to initiate registrational studies in H2 2021



Dementia Program Comprehensive Strategy

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

Treatment Settings Spectrum

Hospitals/EDs

Assisted Living/ Nursing Homes

Community/At Home

*As needed

The Significant Need for Managing Dementia Related Agitation

Click to view the Dementia related Agitation video

BXCL701

Potential First-in-Class Oral IO Therapy

BXCL701 Clinical Development Strategy

Encouraging signals of activity 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

Topline efficacy data expected in Q2 2021

Plans to present results at major medical conference

* CPI: Check Point Inhibitors

bioxcel therapeutics

2021

Our Transformative Year Ahead

Key Clinical and Commercial Catalysts for 2021

Strong cash position $213M to fund key milestones*

NEUROSCIENCE – BXCL501

Schizophrenia & Bipolar:

- NDA submission planned in Q1 2021

Dementia:

- ✓ **Announced positive data from the Phase 1b/2 TRANQUILITY trial**
- EOP2 meeting with the FDA in H1 2021
- Initiate registrational trial(s) in H2 2021

Opioid Withdrawal Symptoms:

- Phase 1b/2 RELEASE trial topline data expected in Q1 2021

Delirium:

- Initiating Phase 2 trial in Q1 2021

COMMERCIAL STRATEGY

- Building commercial and medical teams to prepare for anticipated BXCL501 commercialization

IMMUNO-ONCOLOGY – BXCL701

Aggressive Form of Prostate Cancer (cold tumor):

- Topline efficacy data readout expected mid-2021

Solid Hot Tumors – Basket Trial:

- Topline efficacy data readout expected mid-2021

*This amount is preliminary, has not been audited and is subject to change upon completion of the Company's audited financial statements for the year ended December 31, 2020.

Thank You!

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