NASDAQ: BTAI

Developing and Commercializing Innovative Medicines Discovered using AI

December 2020

Forward-Looking Statements

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BioXcel Therapeutics: A Fully Integrated Discovery and Development Organization Powered by AI

AI-Powered Drug Development

- Identifies novel opportunities for clinical stage compounds
- Improves R&D economics
- Potentially reduces development timelines

Neuro Program

BXCL501—Sublingual Thin Film for Acute Treatment of Agitation

- Successful Phase 3 schizophrenia/bipolar trials (SERENITY I & II); Initiated rolling submission of NDA with FDA
- Phase 1b/2 dementia trial (TRANQUILITY): initiated third dose cohort of 90 mcg
- Phase 1b/2 opioid withdrawal trial (RELEASE) initiated; readout expected Q1 2021
- Phase 2 delirium trial expected to initiate within the next several months

Immuno-oncology Program

BXCL701—Targeting Rare Cancers

- Phase 1b/2 double combo trial in tNEPC and CRPC ongoing; Phase 2 efficacy portion initiated
- MD Anderson led Phase 2 basket trial in advanced solid tumors ongoing

Complete NDA submission for BXCL501 expected in Q1 2021

AI Platform – Greater Predictability and Efficiency

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

Multiple Candidates Screened By A I

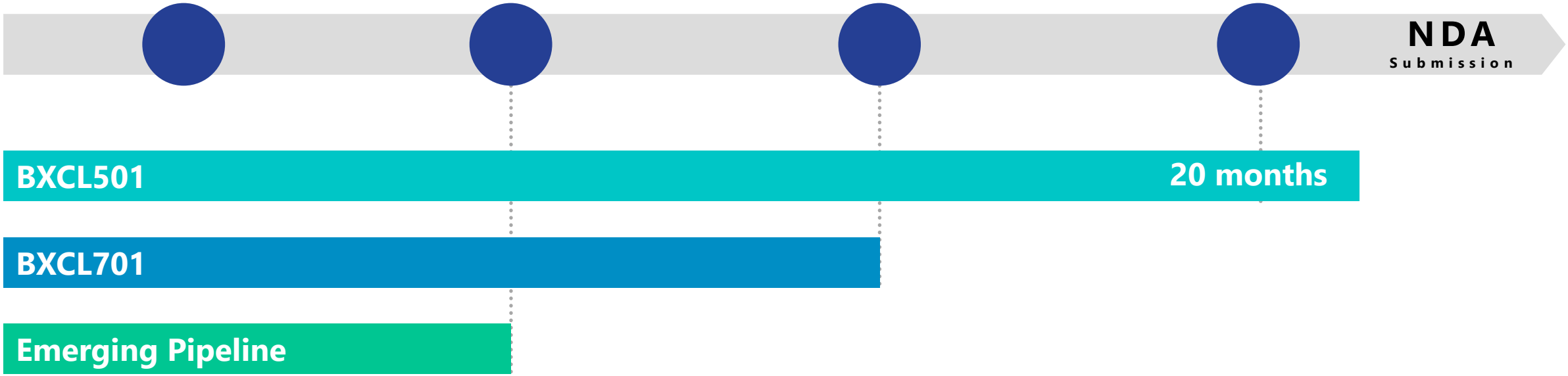
Selection of Best Candidates

Translational Team

Candidate Validation

Clinical Development Team & Regulatory Team

Human Proof of Concept & Registration Trials



AI Pipeline – Multiple Opportunities With Each Candidate

Neuropsychiatry	
BXCL501	
Acute agitation in schizophrenia/bipolar	**SERENITY I & II Trials (Phase 3 Complete)**
Acute agitation in dementia	**TRANQUILITY Trial (Phase 1b/2)**
Opioid withdrawal symptoms	**RELEASE Trial (Phase 1b/2)**
Agitation in delirium	**Phase 2 initiation expected**
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 (Double Combination)**
Basket trial – hot tumors (MD Anderson Led)	**Phase 2 (Double Combination)**

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

BXCL501:

Potential First-in-Class Sublingual Thin Film Dexmedetomidine (Dex) for Acute Treatment of Agitation

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 150 million people worldwide[1] with schizophrenia, bipolar disorder, dementia, delirium and opioid use disorder
  - Over 13M patients in the U.S. experience agitation
  - Multi-billion dollar financial burden
- Current treatment options are suboptimal
  - Physically restraining patients
  - Over-sedating therapies such as antipsychotic and benzodiazepines
  - Antipsychotic drugs have black box warning for elderly
- BXCL501 offers a novel mechanism and a highly differentiated approach

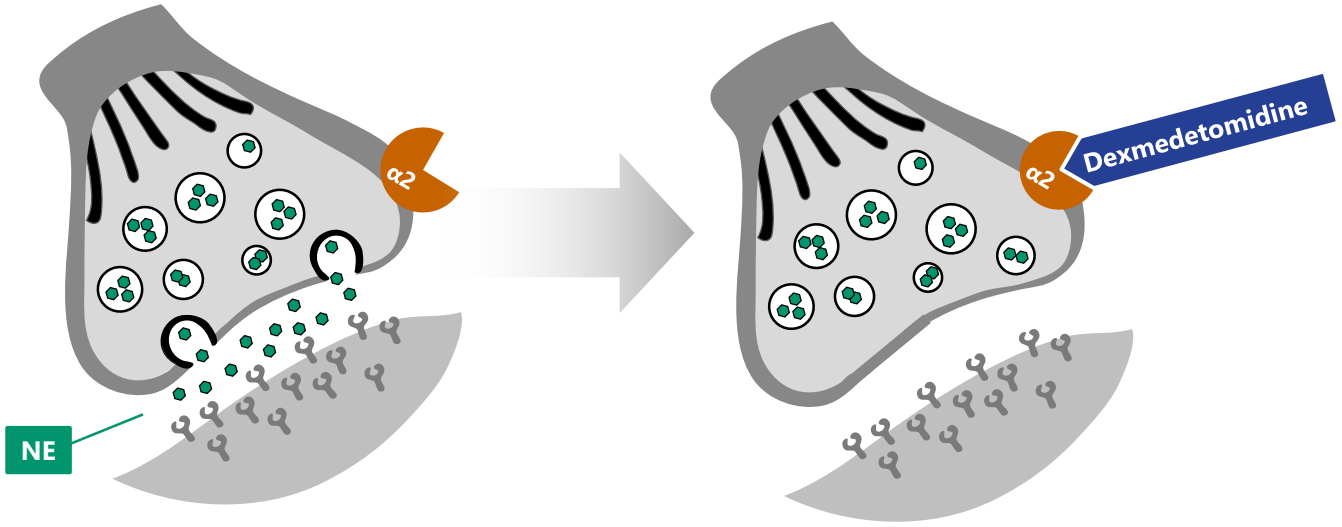
Fast Track Designation

BXCL501 for treatment of mild to moderate acute agitation associated with schizophrenia, bipolar disorder, or dementia

1. Internal company estimates.

BXCL501: Proprietary Sublingual Thin Film of Dex* Designed to Block Driver of Agitation

Dexmedetomidine MoA



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

Norepinephrine (NE)

*Dexmedetomidine

Novel Mechanism May Directly Target Causal Agitation

- Dex activates at the alpha-2a receptor preventing the release of norepinephrine

Highly Differentiated from Current Treatments

- ✔ Easy to administer, sublingual or buccal
- ✔ Non-traumatic
- ✔ Rapid onset of action, without excessive sedation (observed in clinical studies)
- ✔ Non-invasive
- ✔ Self-administered by patients

Patients Successfully Self Administered Film in Trials

Proprietary, Orally Dissolving, Sublingual or Buccal Thin Film Formulation

- Muco-adhesion properties designed for optimizing compliance
- Adaptable technology enables broad dose range
- Flexible for potential combination of multiple drugs on a single film
- U.S. patent (No. 10,792,246) issued; IP protection expected until 2039

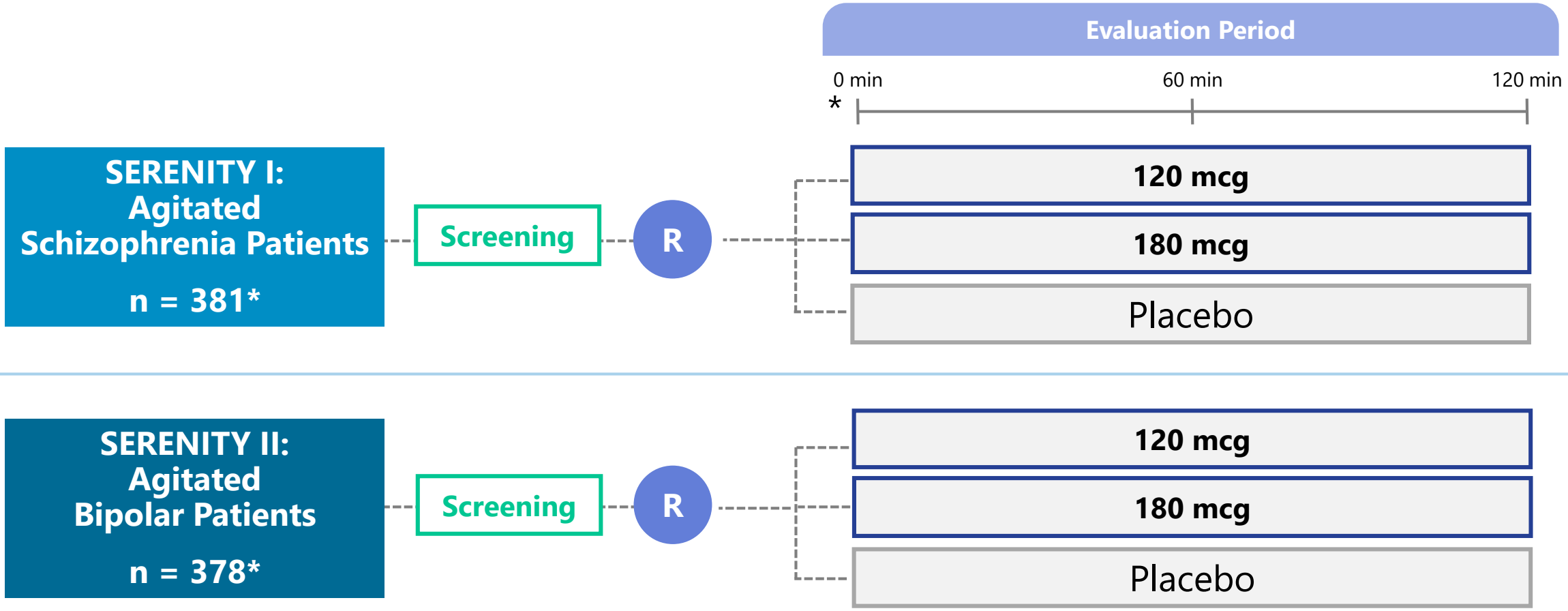
Transitioned to Registrational Drug Product Process

- Manufacturing Phase 3/registrational batches
- Commercial scale-up planned for product launch

Robust Treatment Effect Observed in Two Phase 3 Studies

- ✓ Highly statistically significant improvements in PEC score observed vs. placebo ($p<0.0001$) at two hours in the SERENITY trials for both doses tested
- ✓ Statistically significant improvements in PEC score observed as early as 20 minutes after treatment
- ✓ All exploratory endpoints demonstrated statistically significant and clinically meaningful reductions in agitation measures that were durable
- ✓ BXCL501 was well tolerated with no serious adverse events
- ✓ Complete NDA submission to U.S. FDA planned for Q1 2021

SERENITY I & II: Two Pivotal Phase 3 Trials of BXCL501



Primary Endpoint: Change from Baseline in PEC Score (PANSS-Excitatory Component) at 2 Hours
Secondary Endpoint: Earliest Time Where an Effect on Agitation is Apparent

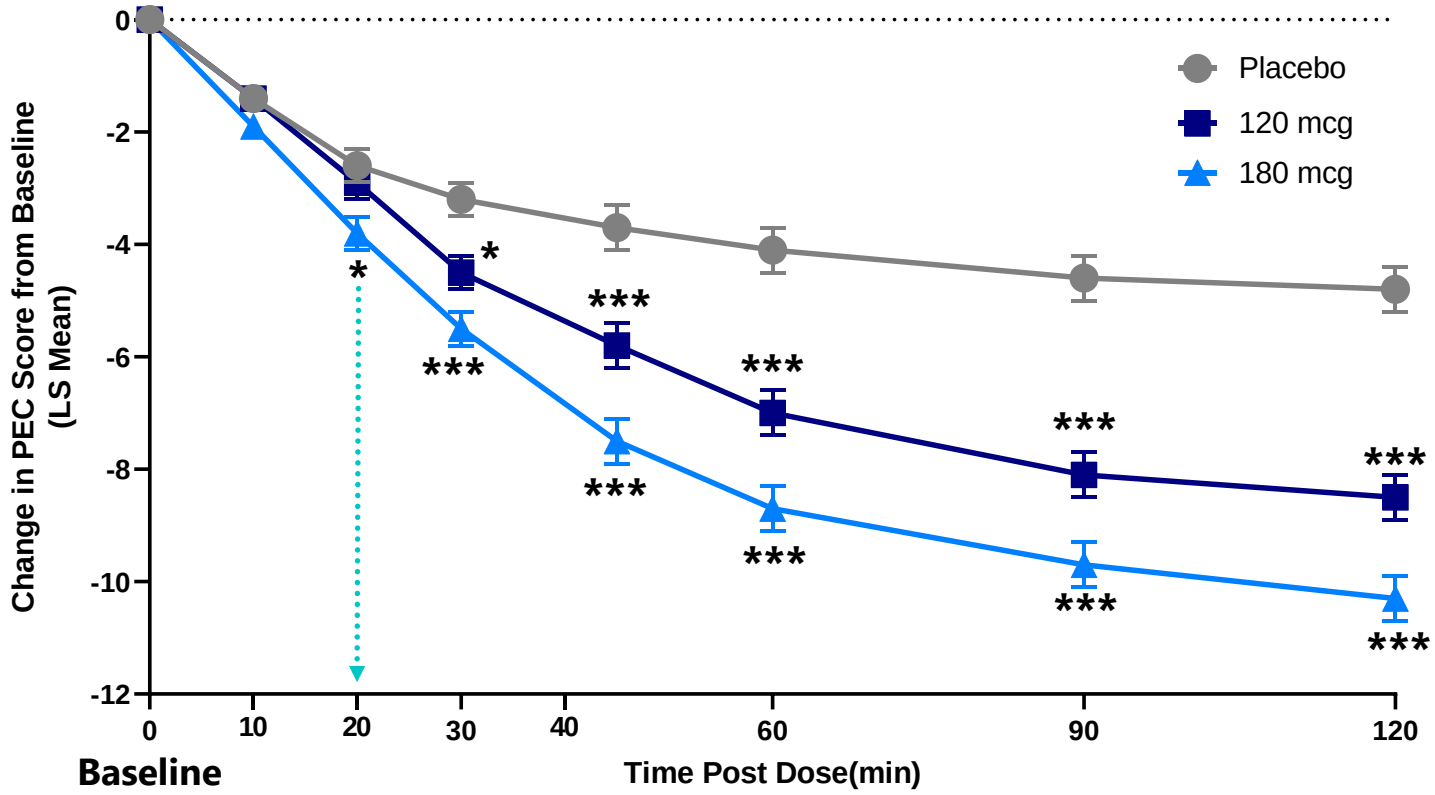
* Patients Dosed

SERENITY I & II: Demographics and Baseline Characteristics

		180 mcg BXCL501		120 mcg BXCL501		Placebo		Overall	
		SERENITY I (N=126)	SERENITY II (N=126)	SERENITY I (N=129)	SERENITY II (N=126)	SERENITY I (N=126)	SERENITY II (N=126)	SERENITY I (N=381)	SERENITY II (N=378)
Mean age (SD)		46.0 (11.91)	45.9 (11.30)	45.7 (11.32)	46.1 (11.53)	45.1 (11.13)	44.8 (12.05)	45.6 (11.43)	45.6 (11.61)
Female N (%)		44 (34.9)	67 (53.2)	52 (40.3)	67 (53.2)	44 (34.9)	73 (57.9)	140 (36.7)	207 (54.8)
Race (% white/ % non-white)		16.7/83.3	38.9 /61.1	25.6/74.4	44.4/55.6	16.7/83.3	39.7/60.3	19.7/80.3	41.0/59
BMI		32.53 (7.9)	33.27 (8.7)	31.24 (7.6)	31.62 (8.0)	32.56 (7.4)	32.50 (7.4)	32.10 (7.6)	32.46 (8.0)
Diagnosis	Schizophrenia	80.2%	-	87.6%	-	85.7%	-	84.5%	-
	Schizoaffective	19.8%	-	12.4%	-	14.3%	-	15.5%	-
	Depressed	-	22%	-	16%	-	1%	-	20%
	Hypomania	-	4%	-	11%	-	8%	-	8%
	Mania	-	47%	-	46%	-	50%	-	48%
	Mixed Episodes	-	24%	-	21%	-	18%	-	21%
	Unspecified	-	3%	-	6%	-	4%	-	4%
Baseline PEC means		17.6	18	17.5	18	17.6	17.9	Range (14 – 27)	Range (14 – 30)

SERENITY I: 98% of randomized patients completed trial; SERENITY II: 95% of randomized patients completed trial

SERENITY I: Rapid Onset of Action Observed



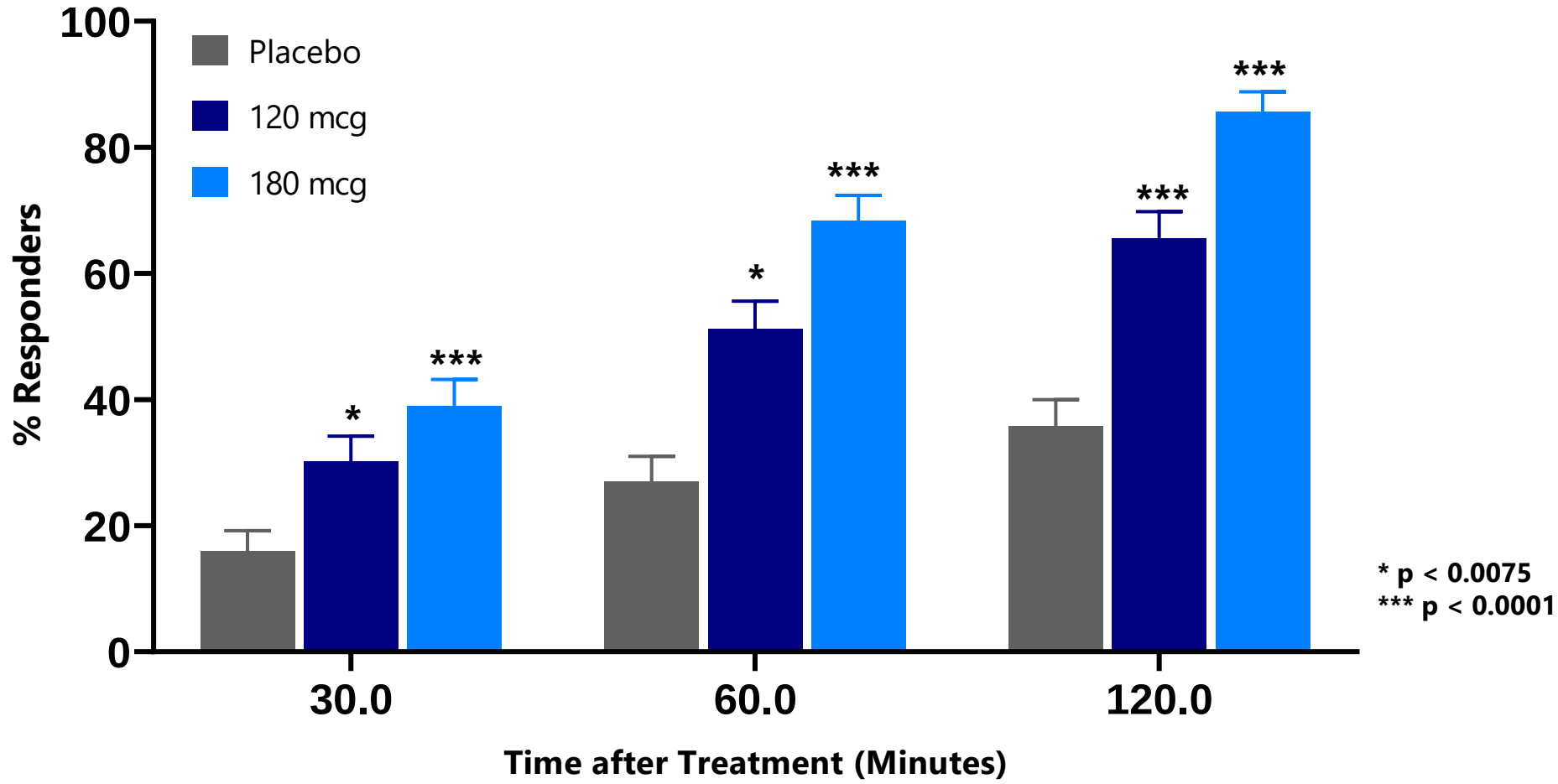
* p < 0.025
*** p < 0.0001

	Endpoint (120 min)	Placebo	120 mcg	180 mcg
Primary Endpoint at 120 Min	PEC Total score Change from Baseline	-4.8	-8.5 ***	-10.3 ***
	Response °	34%	67% ***	87% ***

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

SERENITY I: Clinically Meaningful Improvement Confirmed by CGI-I

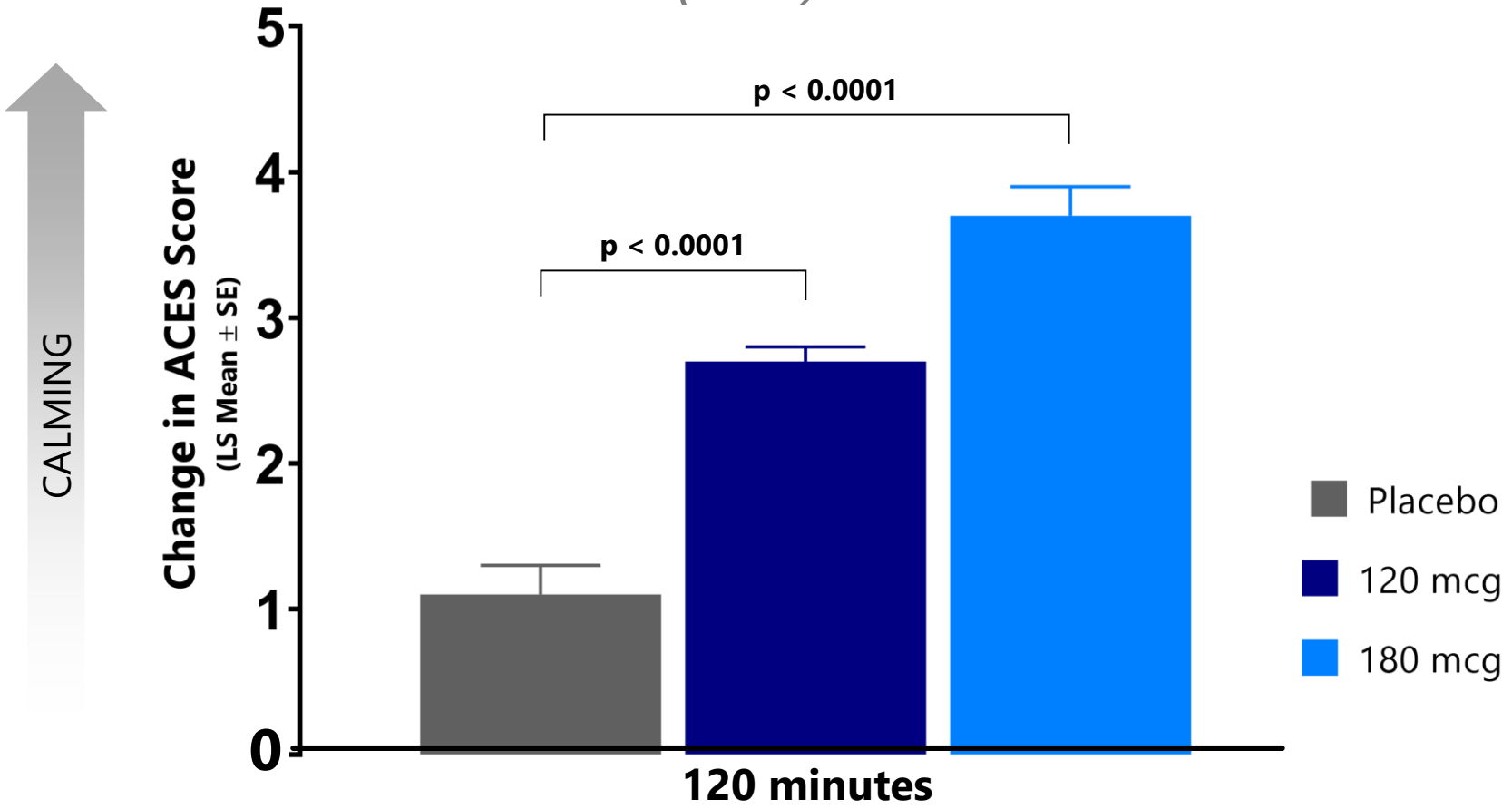
Measured by Clinical Global Impression – Improvement Scale (CGI-I)



The Clinical Global Impression scale – Improvement (CGI-I) is a 7-point scale. Ratings of (1) Very Much, or (2) Much Improved were considered Responders.
ITT analysis

SERENITY I: Independent Confirmation of Calming by ACES

Agitation and Calmness Evaluation Scale (ACES)

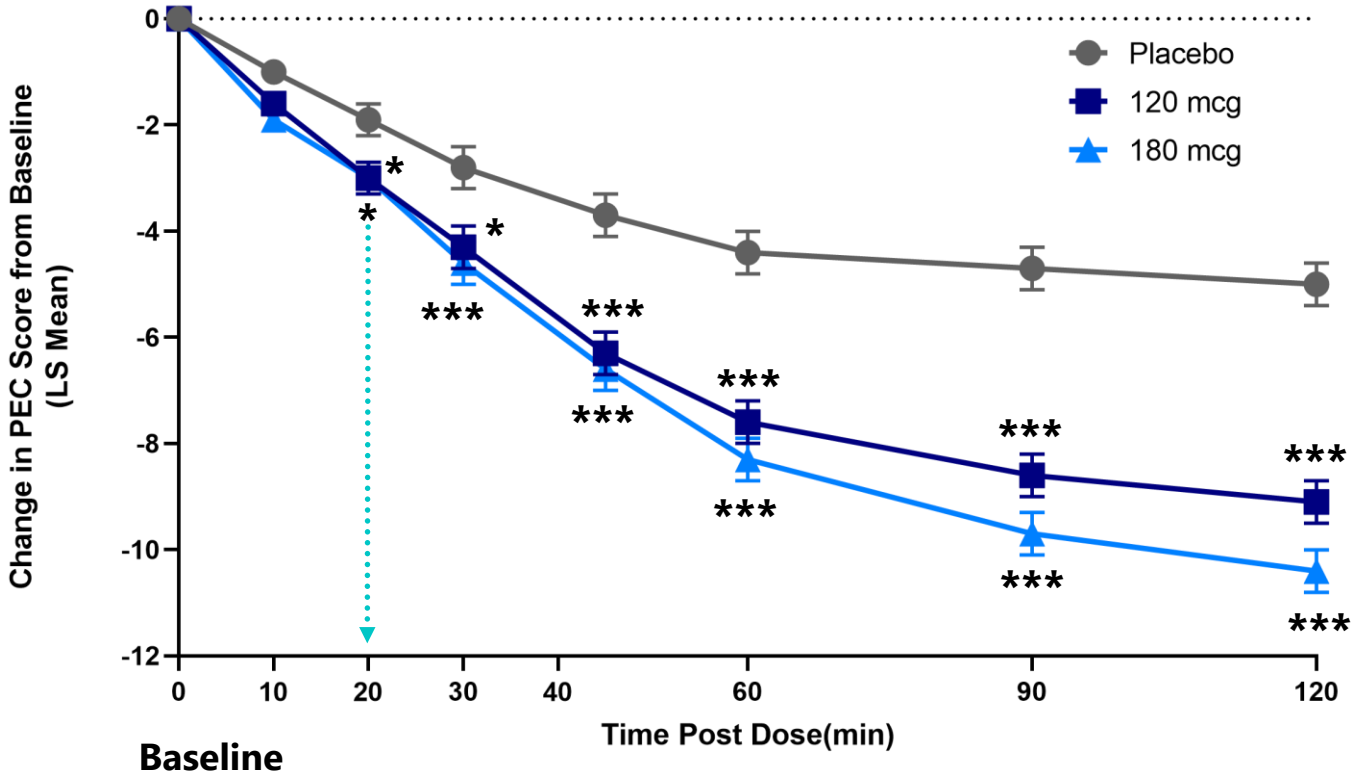


Significant calming observed at both doses

The ACES consists of a single item that rates overall agitation and sedation at the time of evaluation, where 1 indicates marked agitation; 2, moderate agitation; 3, mild agitation; 4, normal behavior; 5, mild calmness; 6, moderate calmness; 7, marked calmness; 8, deep sleep; and 9, unarousable.

ITT analysis

SERENITY II: Rapid Onset of Action Observed



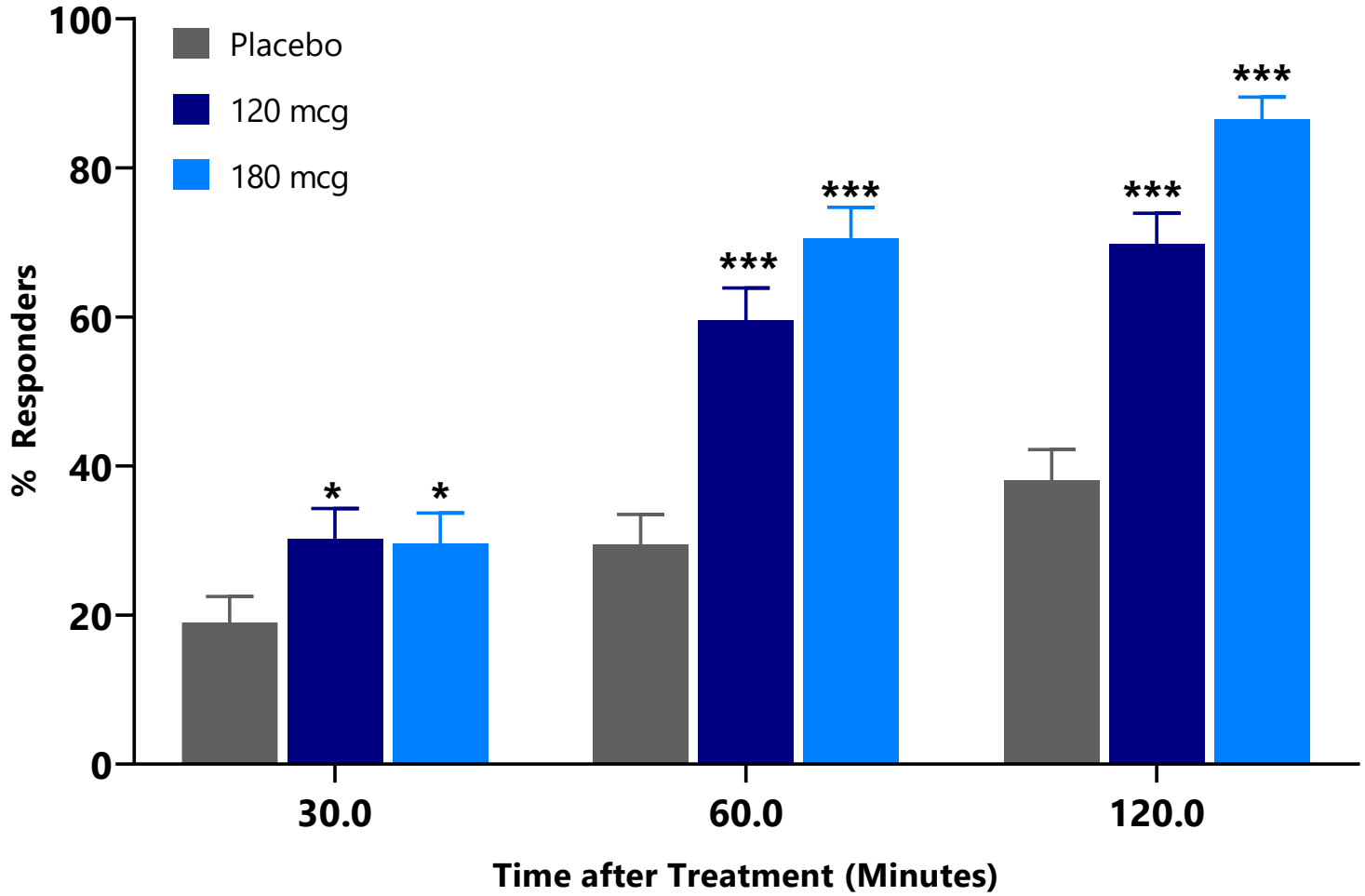
*** p < 0.025**
***** p < 0.0001**

Time = 120 Min (Primary Endpoint)	Endpoint (120 min)	Placebo	120 mcg	180 mcg
	Primary: 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

SERENITY II: Clinically Meaningful Improvement Confirmed by CGI-I

Measured by Clinical Global Impression – Improvement Scale (CGI-I)

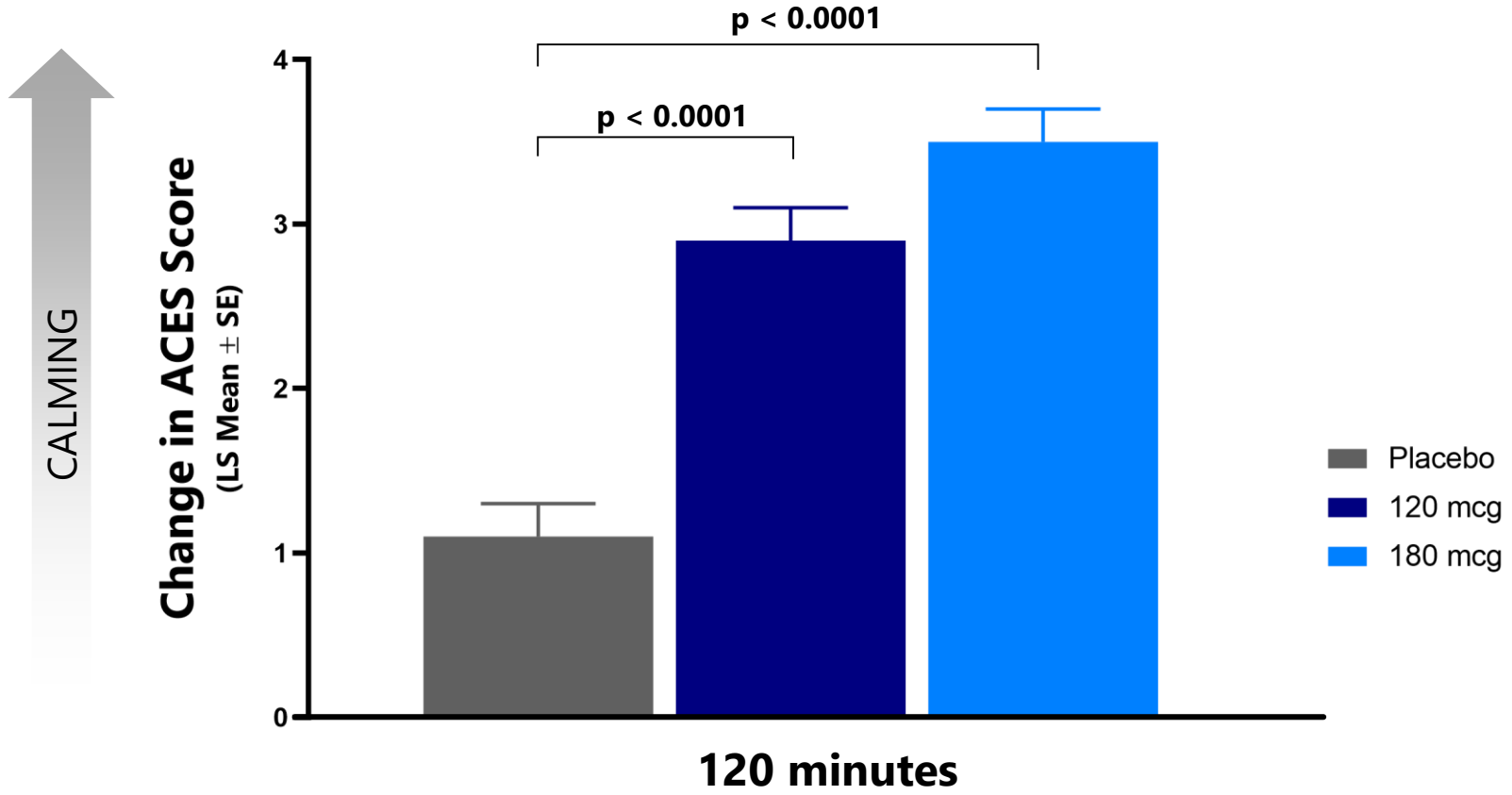


* p = 0.0567
** p = 0.0066
*** p < 0.0001

The Clinical Global Impression scale – Improvement (CGI-I) is a 7-point scale. Ratings of (1) Very Much, or (2) Much Improved were considered Responders.
ITT analysis

SERENITY II: Independent Confirmation of Calming by ACES

Agitation and Calmness Evaluation Scale (ACES)



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ITT analysis

BXCL501 Well Tolerated with No Serious Adverse Events

Tolerability Similar in Both SERENITY Trials

		180 mcg BXCL501 (N=252)	120 mcg BXCL501 (N=255)	Placebo (N=252)
Somnolence	Mild	40 (15.9)	43 (16.9)	15 (6.0)
	Moderate	16 (6.3)	11 (4.3)	1 (0.4)
Dizziness	Mild	13 (5.2)	7 (2.7)	2 (0.8)
	Moderate	2 (0.8)	3 (1.2)	0
Hypotension	Mild	10 (4.0)	10 (3.9)	0
	Moderate	3 (1.2)	4 (1.6)	0
Orthostatic hypotension	Mild	9 (3.6)	7 (2.7)	1 (0.4)
	Moderate	4 (1.6)	0	0
Hypoaesthesia oral		12 (4.8)	7 (2.7)	1 (0.4)
Dry mouth		11 (4.4)	19 (7.5)	3 (1.2)
Nausea		7 (2.8)	6 (2.4)	4 (1.6)
Headache		6 (2.4)	12 (4.7)	12 (4.8)
Paraesthesia oral		6 (2.4)	7 (2.7)	1 (0.4)

All subjects self-administered the sublingual film

Treatment Emergent Adverse Events (TEAEs) with >2% incidence rate in one or more treatment groups are included, sorted by decreasing frequency in the order of 180 ug BXCL501, 120 ug BXCL501, Placebo. Subjects counted once at highest severity within each term based on MedDRA (Medical Dictionary for Regulatory Activities) version 23.0

Preparing for Potential Commercialization

Strong Commercial and Medical Leadership

William Kane –
Chief Commercial Officer

Allergan · Pfizer · SEPRACOR

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

cognivue · Pfizer · NOVARTIS

Building Cross-Functional Team

- Core Team: Q3 ‘20
- Medical: MSL team of 20, including Payer MSLs, launching in Q1 ‘21
- Market Access: Account Management Team on board mid ‘21
- Sales: 75-100 representative sales force at launch to cover high volume institutions

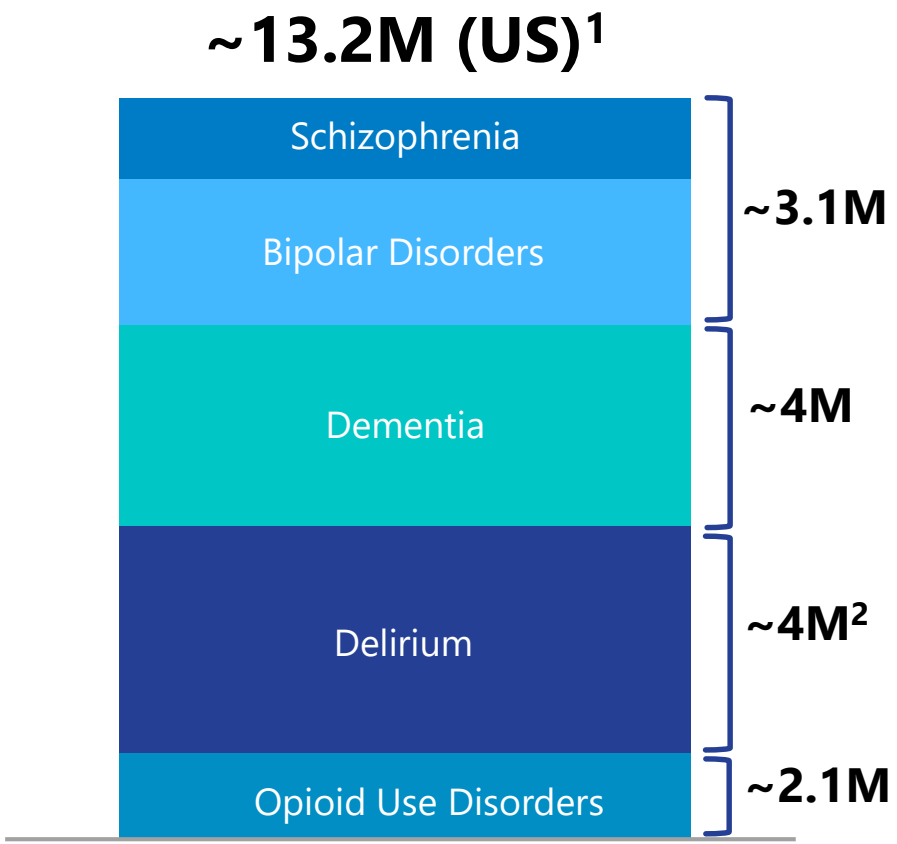
Commercialization Outside U.S.

- Seeking regional partners for Japan and Europe

Expanding Potential Agitation Indications for BXCL501

Upcoming Milestones

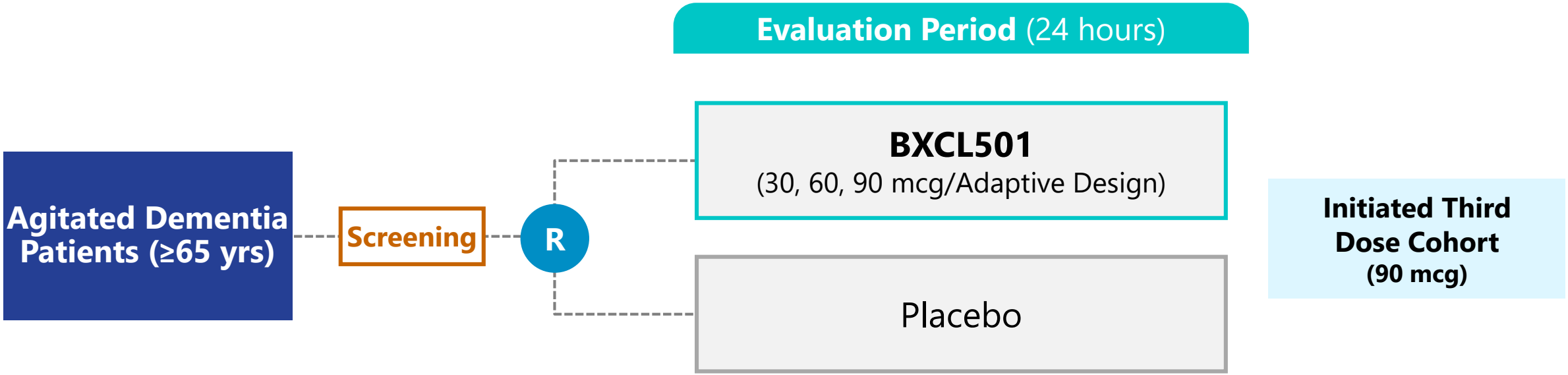
Indication	Upcoming milestones
Schizophrenia & bipolar disorders	• NDA submission expected Q1 2021
Dementia	• Initiated third dose cohort (90 mcg)
Opioid withdrawal symptoms	• Topline readout expected Q1 2021
Delirium (including COVID-19 patients)	• Phase 2 trial expected to initiate within the next several months
PTSD, traumatic brain injury, alcohol withdrawal and treatment of phobias	• Evaluating commercial opportunity



1. Internal company estimates based on analysis of primary market research, prescription database, and published data.
2. Includes patients with agitated delirium in ICU, medical and surgical wards.

TRANQUILITY: Phase 1b/2 Proof-of-Concept Trial in Dementia

Goal is to Identify Tolerable and Effective Dose(s) for Late-Stage Trial



Key Inclusion Criteria

- Dementia (all forms including AD)
- Clinically significant agitation

Exploratory Efficacy Endpoints

- PAS: Pittsburgh Agitation Scale
- PEC: PANSS Excitatory Component
- CMAI: Cohen-Mansfield Agitation Inventory (modified)

BXCL501 Potential Across the Spectrum of Agitation in Dementia

Dementia Progression	Pre-Agitation	Acute/Episodic Agitation	Sub-chronic Agitation	Chronic Agitation
BXCL501	Wearable + PRN	PRN	PRN	Daily (BXCL501 + combination)

Emergency Departments
General
Psychiatric

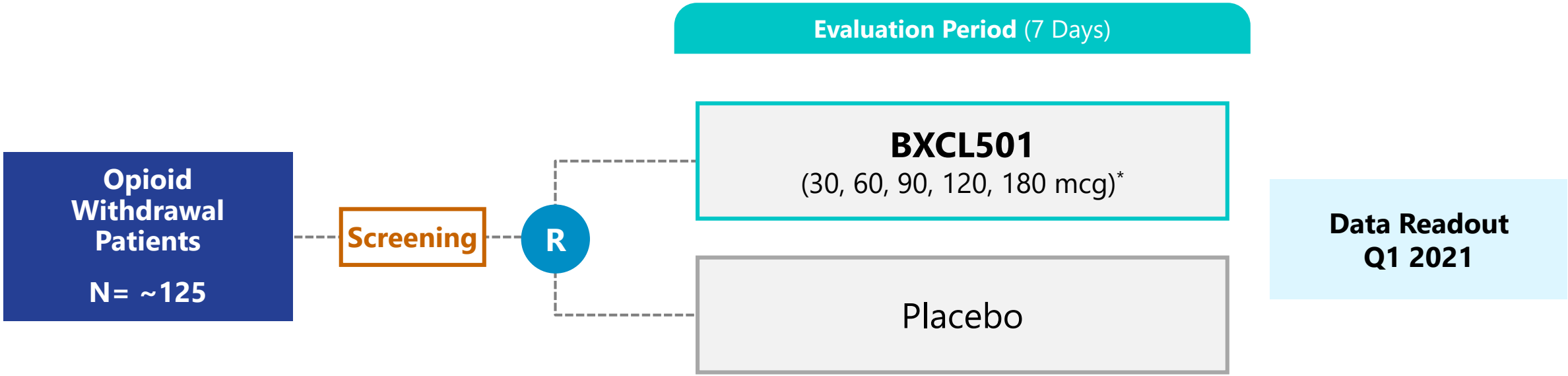
Hospitals
Medical + Neuro + Psych Wards
Psychiatric Hospitals
Geriatric Units

Nursing Homes
Assisted Living

Medical Clinics
Neurology/Mental Health
Crisis Care

RELEASE Phase 1b/2 Trial Initiated – Opioid Withdrawal Symptoms

Sub-Chronic Usage



Key Inclusion Criteria and Design

- Street/prescription opiate abusers with signs and symptoms of opiate withdrawal before entry
- Double-blinded, placebo-controlled dose escalation design

Endpoints

- Pharmacokinetics, Safety and Tolerability
- COWS: Clinical Opiate Withdrawal Scale
- SOWS: Short Opiate Withdrawal Scale of Gossop

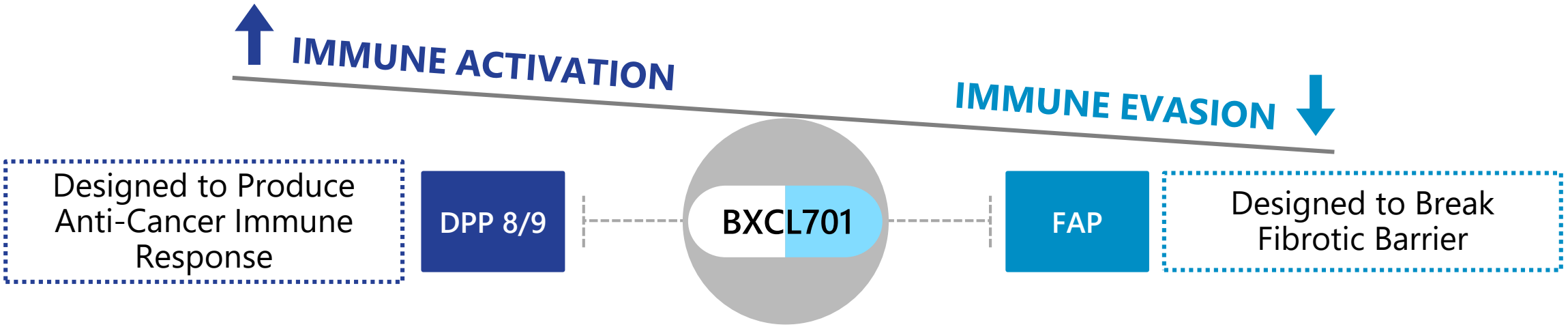
*Administered twice daily, approximately 12 hours apart

BXCL701

Potential First-in-Class 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 in difficult-to-treat tumors observed in both trials

1

Prostate Cancer—tNEPC and CRPC (Cold Tumors):

Phase 1b/2 trial of BXCL701 and KEYTRUDA

2

Solid Tumors Responsive (Hot Tumors) to CPIs*:

Open-label Phase 2 basket trial led by MD Anderson

Orphan Drug Designations

FDA

- Pancreatic Cancer
- Melanoma
- Acute Myeloid Leukemia

* CPI: Check Point Inhibitors

BXCL701 Program Timeline

	H1 2020	H2 2020	Mid-2021
Prostate Cancer (tNEPC and CRPC)	Phase 2 Efficacy Portion Initiated	Initial Safety & Efficacy Data Presented	Topline Efficacy Data Readout Expected
Solid Tumors Responsive to CPIs*	Phase 2 Basket Trial Initiated	Initial Efficacy Data Presented	Topline Efficacy Data Readout Expected

*MD Anderson Led IST

Thank You!

Dr. Vimal Mehta, CEO
BioXcel Therapeutics, New Haven, CT 06511
vmehta@bioxceltherapeutics.com