bioxcel therapeutics

(NASDAQ: BTAI)

BXCL501 Phase 1b Results Conference Call

July 22, 2019

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, the commencement of clinical trials, the availability and results of data from clinical trials, BioXcel Therapeutic, Inc.'s (“BTI”) submission of its first New Drug Application with the FDA 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 BTI's current expectations and various assumptions. BTI believes there is a reasonable basis for its expectations and beliefs, but they are inherently uncertain.

BTI 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; it ability to receive regulatory approval for its product candidates; its ability to enroll patients in its clinical trials; 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; 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 period ended March 31, 2019 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.

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 BTI 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 BTI's views as of any date subsequent to the date of this presentation.

Agenda

BXCL501 Phase 1b Trial Results

Overview and Summary	**Vimal Mehta, Ph.D., CEO & Founder**
BXCL501 Phase 1b Trial Design and Results	**Rob Risinger, M.D., VP, Clinical Development**
Q&A	**BioXcel Therapeutics Team**
Corporate Outlook & Closing Remarks	**Vimal Mehta, Ph.D., CEO & Founder**

BioXcel Therapeutics Team

- ✓ **Vincent O'Neill, M.D.**, *Chief Medical Officer*
- ✓ **Frank Yocca, Ph.D.,** *Chief Scientific Officer*
- ✓ **Chetan Lathia, Ph.D.,** *SVP and Head, Translational Medicine, Clinical Pharmacology and Regulatory Affairs*
- ✓ **Richard Steinhart,** *Chief Financial Officer*

Overview and Summary

Vimal Mehta, Ph.D., CEO & Founder

BXCL501 Phase 1b Trial

Summary of Results

- BXCL501: a potential first-in-class, proprietary sublingual thin film of dexmedetomidine, a selective alpha-2a receptor agonist (a novel mechanism of action).
- Clinically meaningful, rapid and durable reductions in PEC score in acute treatment of agitation in schizophrenia patients.
- Statistically significant mean reduction in PEC score at two hours compared to placebo following a single dose of 180 mcg ($p<0.0001$), with rapid and durable effects maintained for 4 to 6 hours across multiple dose strengths.
- Approximately 90% of patients met response criteria at 180 mcg with a reduction in PEC score (≥40%).
- Well-tolerated with no serious or severe adverse events across the entire dose range.
- Data support progressing BXCL501 program potentially to Phase 3 pivotal trials, subject to discussions with FDA.

About Agitation

Overview

- Common and costly phenomenon associated with number of psychiatric conditions
  - 8.3 million suffer from agitation each year in the US
  - $40 billion per year health care burden
- Consensus guidelines recommend non-coercive management strategies to protect therapeutic alliance between patients and healthcare providers
- Unmet medical need: rapid symptom relief with non-invasive approach

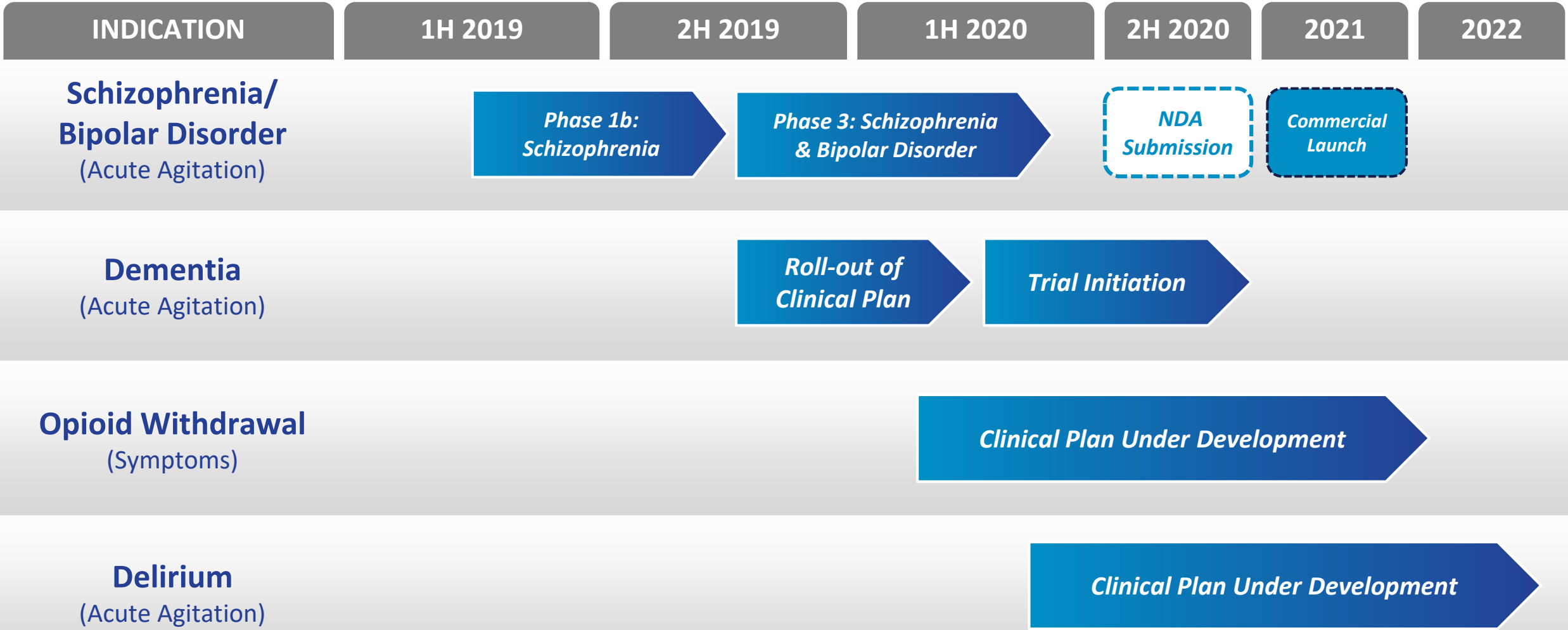
~19M At Risk

8.3M **Suffer Agitation**

3M - 5M **Mild to Moderate Agitation**

BXCL501: Large Market Potential

Anticipated Timeline: First NDA Submission in 2H 2020



BXCL501 Phase 1b Trial Design and Results

Rob Risinger, M.D., VP, Clinical Development

BXCL501

Sublingual Thin Film Dexmedetomidine (Dex) for Acute Treatment of Agitation

Unmet Need

Current **Treatments are Suboptimal**:

- **Dementia**: Antipsychotic drugs (black-box warning) for elderly
- **Psychiatric**: Invasive with severe side effects

Consensus Opinion*

- **Non-invasive**
- **Calmness without sedation**
- **Easy to administer**
- **Rapid onset**
- **Non-traumatic / non-coercive**
- **Good safety profile**
- **Favorable tolerability**
- **Patient preference**

FDA *Fast Track Designation*

BXCL501: Novel Mechanism of Action (MoA)

✓ Non-Invasive, easy to administer **sublingual thin film** designed for **rapid onset of action**

*1st International Experts' Meeting on Agitation: Conclusions Regarding the Current and Ideal Management Paradigm of Agitation, Frontiers in Psychiatry 2018

BXCL501 Phase 1b Trial

Clinical Trial Design

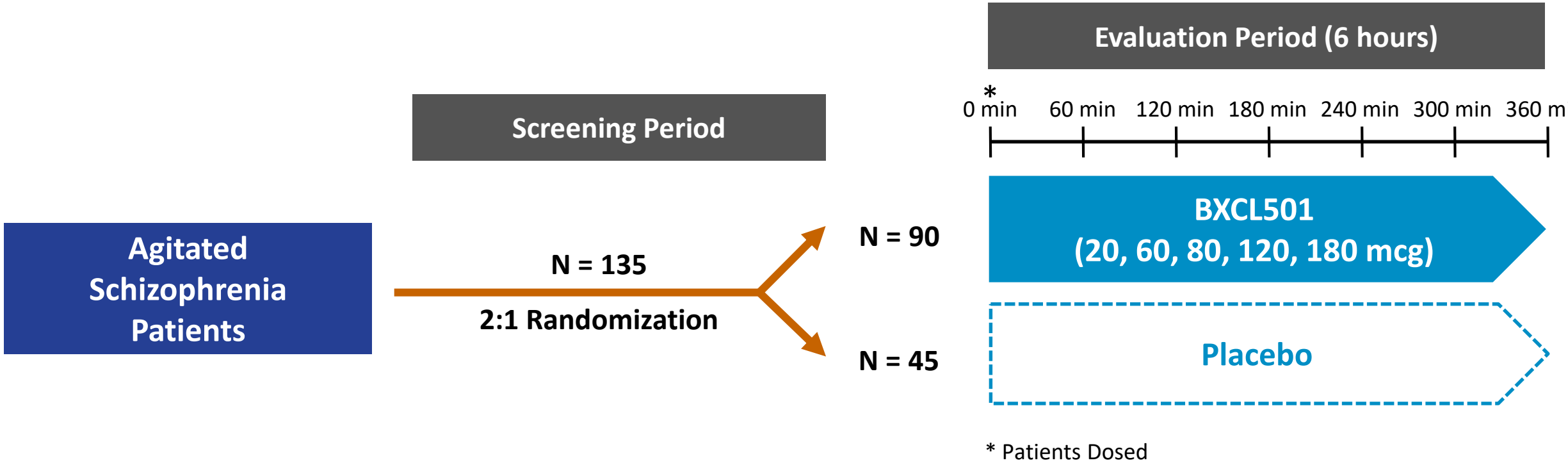
- Phase 1b, randomized, double-blind, placebo-controlled, multi-center, U.S. trial
- N=135 adult patients with confirmed diagnosis of schizophrenia
- 6 hour treatment period
  - Single dose*
- Dose groups (2:1 randomization)
  - BXCL501 (20, 60, 80, 120, and 180 mcg)
  - Placebo

*The lowest dose tested, 20 mcg was repeated in subjects who did not achieve response criterion.

BXCL501 Phase 1b Trial

Clinical Trial Design

Assessing Agitation Episodes in Schizophrenia



* Patients Dosed

Primary Endpoint:
Change from baseline in PEC score (PANSS-Excitatory Component)

✓ Initiated May 2019 →
✓ Completed July 2019

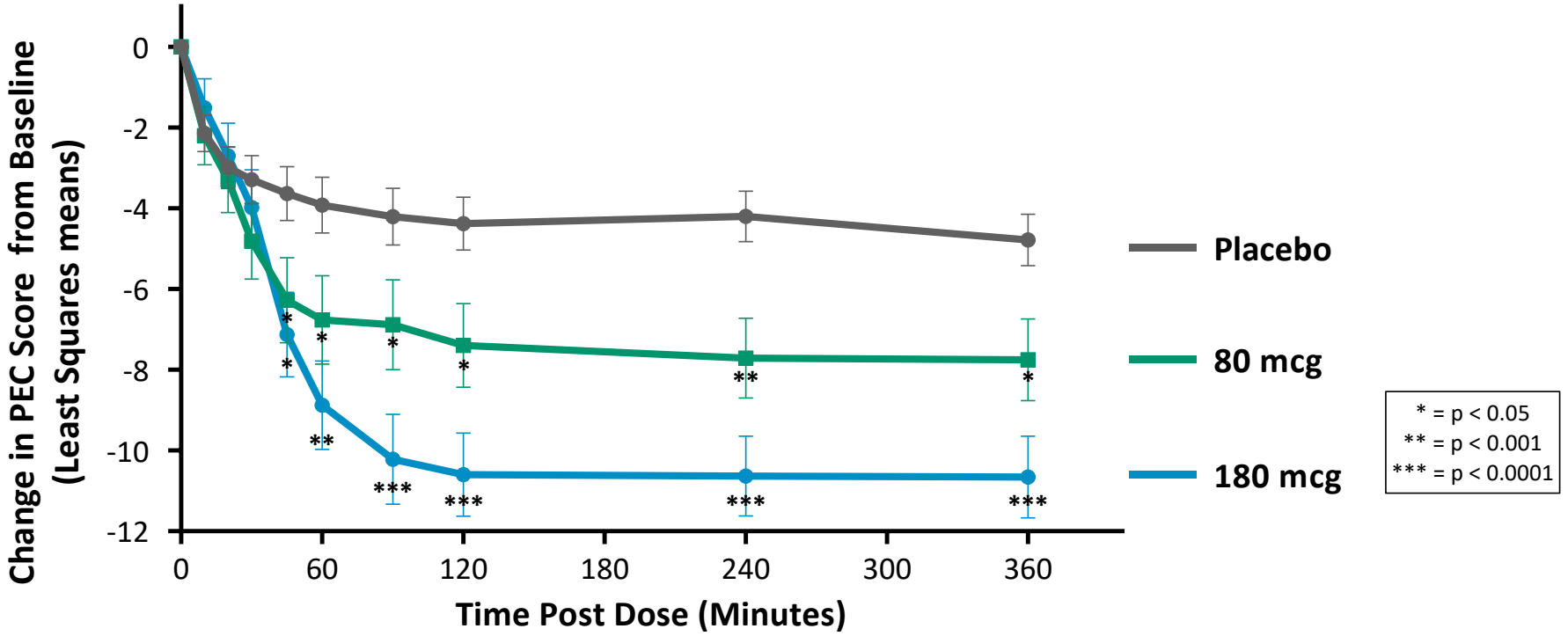
BXCL501 Phase 1b Trial

Demographics and Baseline Characteristics

- Mean age (years): 48.4 for placebo group, 47.2 for BXCL501 group
- – 89 males:46 females
- Mean PEC score at baseline: 18.1 (range 14 – 25) for placebo group, 17.8 (range 14 – 26) for BXCL501 group
- Subjects were on a range of typically prescribed antipsychotics.

BXCL501 Phase 1b

Primary Endpoint: Change in PEC Score from Baseline



		% Responders (Reduction in PEC of ≥ 40%)	Mean Change in PEC Score from Baseline	P-Value
Placebo	N=36	28%	-4.5	
BXCL501 (180 mcg)	N=18	89%	-10.8	< 0.0001
BXCL501 (120 mcg)	N=18	67%	-9.2	0.0003
BXCL501 (80 mcg)	N=18	56%	-7.1	0.0152
BXCL501 (60 mcg)	N=18	39%	-6.0	0.1227

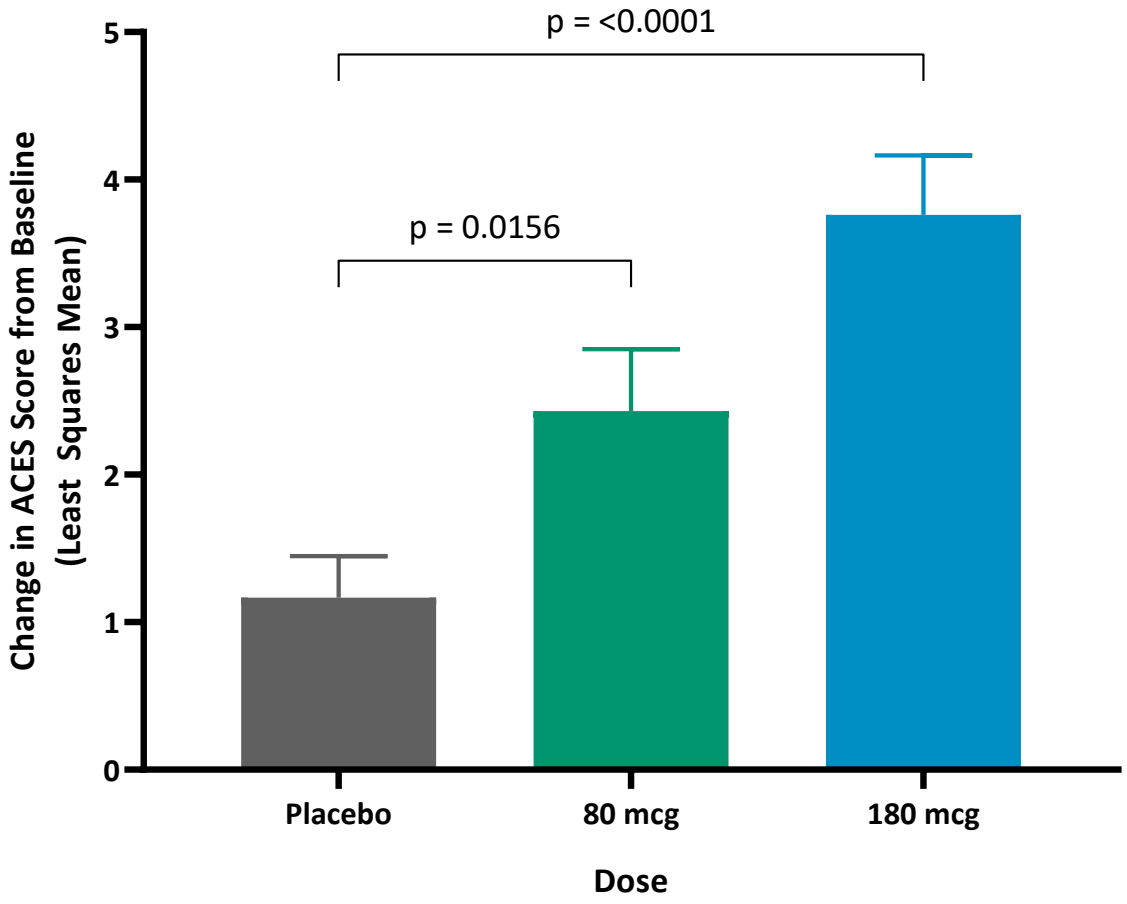
*The lowest dose tested, 20 mcg (not shown) was repeated in subjects who did not achieve response criterion

At Time = 120 Min (Primary Endpoint)

bioxcel therapeutics

BXCL501 Phase 1b

Secondary Evaluation: Change in ACES from Baseline



		Mean Change in ACES Score From Baseline	P-Value
Placebo	N=36	1.20	
BXCL501 (180 mcg)	N=18	3.94	< 0.0001
BXCL501 (120 mcg)	N=18	3.11	0.0005
BXCL501 (80 mcg)	N=18	2.33	0.0156
BXCL501 (60 mcg)	N=18	2.11	0.0750

*The lowest dose tested, 20 mcg (not shown) was repeated in subjects who did not achieve response criterion

BXCL501 Phase 1b Trial

Safety

- BXCL501 was well-tolerated with no serious or severe adverse events across the entire dose range.
- The most common treatment-related adverse events were mild somnolence and dry mouth.
- A maximum tolerated dose was not reached.
- All subjects (100%) were able to self-administer the film and complete the study.

BXCL501 Phase 1b Trial

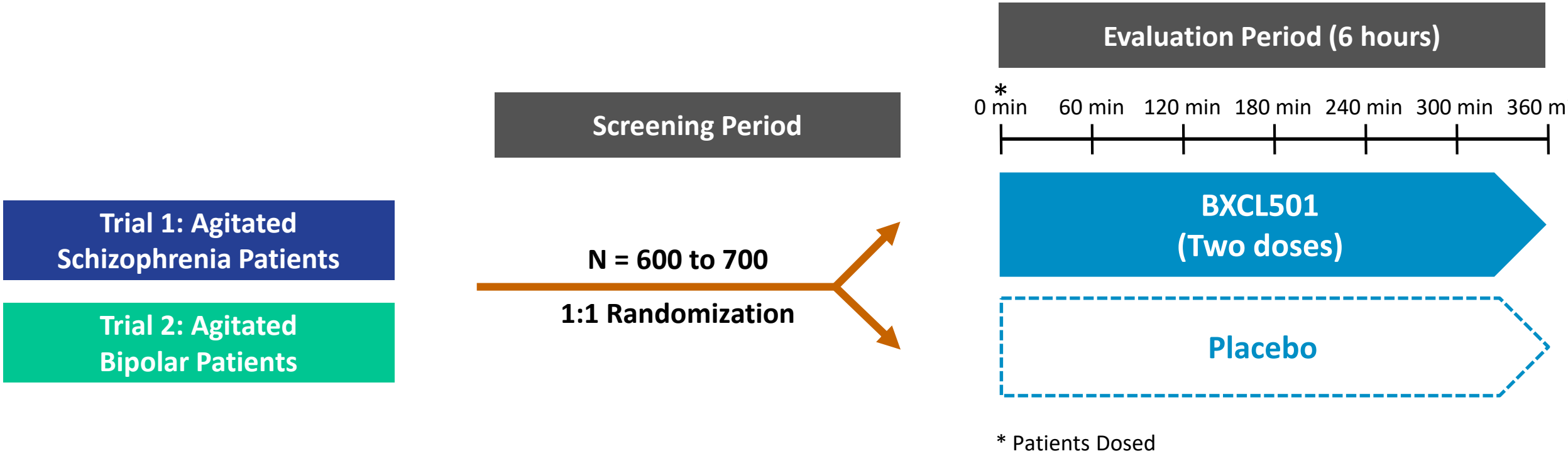
Summary

- Statistically significant improvement in PEC score in the 80, 120, and 180 mcg dose groups for BXCL501 in patients with schizophrenia.
- Calming effect was durable lasting at least 6 hours as evidenced by separation from placebo for 80, 120 and 180 mcg dose groups.
- Well-tolerated across all doses tested.
- A maximum tolerated dose was not observed.
- We believe data support progressing the BXCL501 program to pivotal trials, subject to further discussions with the FDA.

BXCL501 Phase 3 Pivotal Trial

Expected Clinical Trial Design

Assessing Agitation Episodes in Schizophrenia and Bipolar Disorder



* Patients Dosed

Primary Endpoint:
Change from baseline in PEC score (PANSS-Excitatory Component)

Expected to Initiate 4Q 2019

Key Targeted Milestones for Value Creation

On Track for First NDA Submission in 2H 2020

BXCL501

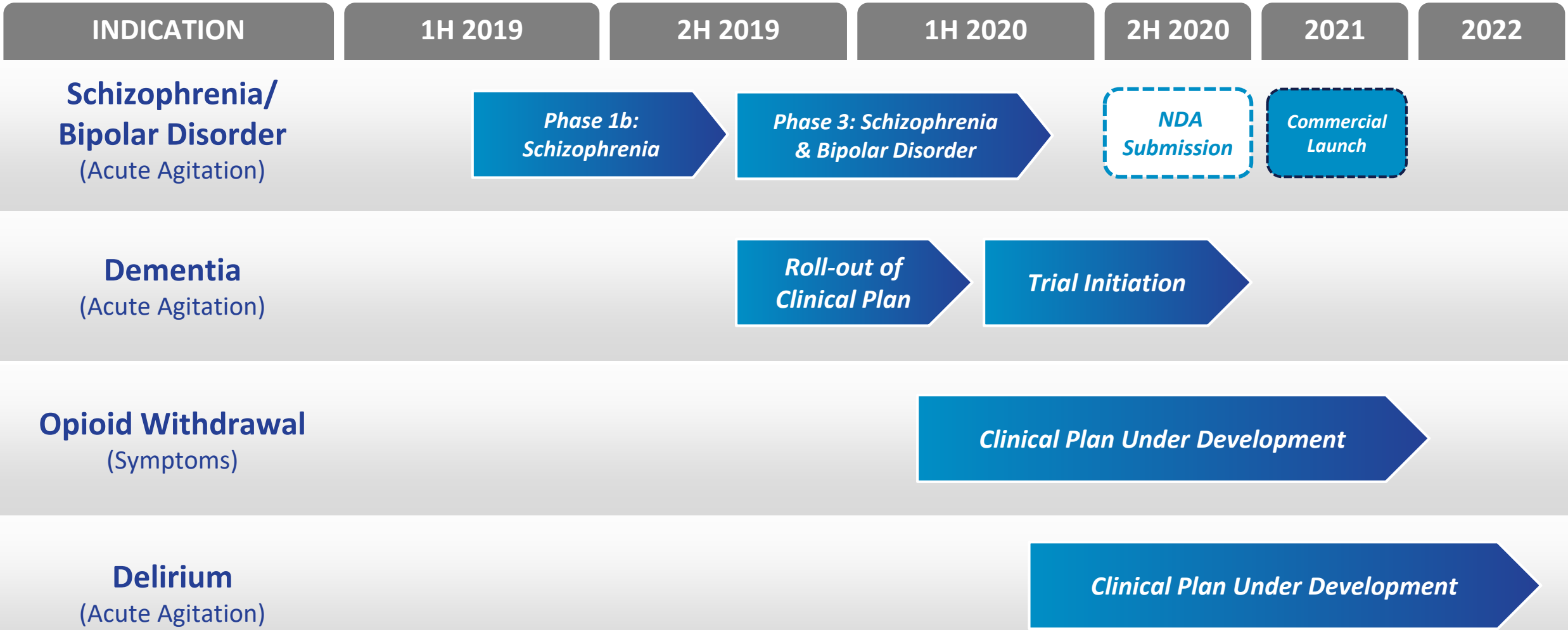
Anticipated Timeline

Schizophrenia / Bipolar Disorder	✓ Phase 1b Trial Initiated (May 2019)	★ ✓ Phase 1b Data Readout (July 22, 2019)	Phase 3 Pivotal Trial Initiation	★ Phase 3 Data Readout	★ First NDA Submission

Development Plans for Agitated Dementia, Opioid Withdrawal Symptoms And Hyperactive Delirium will be Presented Through 2019

BXCL501: Large Market Potential

Anticipated Timeline: First NDA Submission in 2H 2020



Q&A

Corporate Outlook And Closing Remarks

Dr. Vimal Mehta, CEO & Founder

Dr. Vimal Mehta, CEO

BioXcel Therapeutics, New Haven, CT 06511

vmehta@bioxceltherapeutics.com