# UNITED STATES
# SECURITIES AND EXCHANGE COMMISSION

**Washington, D.C. 20549**

# FORM 8-K

**CURRENT REPORT**
**Pursuant to Section 13 or 15(d) of the**
**Securities Exchange Act of 1934**

Date of Report (Date of earliest event reported) **May 20, 2019**

# BioXcel Therapeutics, Inc.

(Exact name of registrant as specified in its charter)

| **Delaware** | **001-38410** | **82-1386754** |
|---|---|---|
| (State or other jurisdiction of incorporation) | (Commission File Number) | (IRS Employer Identification No.) |

**555 Long Wharf Drive**
**New Haven, CT 06511**
(Address of principal executive offices, including Zip Code)

**(475) 238-6837**
(Registrant's telephone number, including area code)

**N/A**
(Former name or former address, if changed since last report)

Check the appropriate box below if the Form 8-K filing is intended to simultaneously satisfy the filing obligation of the registrant under any of the following provisions:

o Written communications pursuant to Rule 425 under the Securities Act (17 CFR 230.425)

o Soliciting material pursuant to Rule 14a-12 under the Exchange Act (17 CFR 240.14a-12)

o Pre-commencement communications pursuant to Rule 14d-2(b) under the Exchange Act (17 CFR 240.14d-2(b))

o Pre-commencement communications pursuant to Rule 13e-4(c) under the Exchange Act (17 CFR 240.13e-4(c))

Securities registered pursuant to Section 12(b) of the Act:

| **Title of each class** | **Trading Symbol(s)** | **Name of each exchange on which registered** |
|---|---|---|
| Common Stock, par value $0.001 | BTAI | The Nasdaq Capital Market |

Indicate by check mark whether the registrant is an emerging growth company as defined in Rule 405 of the Securities Act of 1933 (§230.405 of this chapter) or Rule 12b-2 of the Securities Exchange Act of 1934 (§240.12b-2 of this chapter).

Emerging growth company x

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 13(a) of the Exchange Act. x

**Item 7.01. Regulation FD Disclosure.**

On May 20, 2019, BioXcel Therapeutics, Inc. (the "Company") issued a press release announcing data from its Phase 1 pharmacokinetic (PK) (bioavailability) and safety study of the Company's BXCL501 product candidate and held a live webcast conference call to discuss these results. A copy of the press release is furnished as Exhibit 99.1 hereto and incorporated under this Item 7.01 by reference.

The information in this Item 7.01 of this Current Report on Form 8-K (including Exhibit 99.1) shall not be deemed "filed" for purposes of Section 18 of the Securities Exchange Act of 1934, as amended (the "Exchange Act"), or otherwise subject to the liabilities of that Section, nor shall it be deemed to be incorporated by reference into any filing of the Company under the Securities Act of 1933, as amended, or the Exchange Act, except as expressly set forth by specific reference in such filing.

**Item 8.01. Other Events.**

A copy of the presentation materials discussed during the May 20, 2019 conference call, regarding the data from the Phase 1 pharmacokinetic (PK) (bioavailability) and safety study of the Company's BXCL501 product candidate is attached as Exhibit 99.2 to this current report on Form 8-K and incorporated under this Item 8.01 by reference.

**Item 9.01 Financial Statements and Exhibits.**

(d) Exhibits.

| Exhibit No. | Description |
|---|---|
| 99.1 | BioXcel Therapeutics, Inc. Press Release, May 20, 2019. |
| 99.2 | BioXcel Therapeutics, Inc. Presentation, May 2019. |

**SIGNATURES**

Pursuant to the requirements of the Securities Exchange Act of 1934, the registrant has duly caused this report to be signed on its behalf by the undersigned hereunto duly authorized.

Date: May 20, 2019

**BIOXCEL THERAPEUTICS, INC.**

/s/ Richard Steinhart
Richard Steinhart
Chief Financial Officer

**Exhibit 99.1**

**BioXcel Therapeutics Achieved Targeted Exposures of BXCL501 Designed For Non-Invasive Acute Treatment of Agitation in Neuropsychiatric Diseases**

*Phase 2 efficacy trial in agitated schizophrenia patients anticipated to commence imminently*

*BXCL501 is a potential first-in-class selective alpha-2a receptor agonist, formulated as a proprietary sublingual thin film of dexmedetomidine*

*Targeted exposure levels that were observed to be therapeutic in prior IV Dex study rapidly achieved in preliminary pharmacokinetic profile from Phase 1 study*

*On track to initiate first Phase 3 pivotal trial in 2H 2019 with NDA submission expected in 2020*

*Global development plans in agitated dementia, opioid withdrawal symptoms and hyperactive delirium to expand market will be unveiled through 2019*

*Conference call to discuss results and BXCL501 program advancement on May 20, 2019 at 8.30 ET*

NEW HAVEN, Conn., May 20, 2019 — BioXcel Therapeutics, Inc. (“BTI” or the “Company”) (Nasdaq: BTAI), today announced positive top line data from its Phase 1 pharmacokinetic (PK) (bioavailability) and safety study of BXCL501, a proprietary sublingual thin-film formulation of dexmedetomidine (Dex), for the acute treatment of agitation across multiple neuropsychiatric indications. Administration of BXCL501 in the Phase 1 pharmacokinetic and safety study successfully achieved targeted exposure levels that were observed to be therapeutic in the Company’s prior IV Dex study and BTI intends to advance BXCL501 into a Phase 2 trial to evaluate efficacy in agitated schizophrenia patients. We believe the results from the Phase 2 study are expected to facilitate powering of a planned Phase 3 pivotal trial. BTI is a clinical-stage biopharmaceutical development company utilizing novel artificial intelligence approaches to identify and advance next wave of medicines in neuroscience and immuno-oncology.

The IND-opening Phase 1 study was a double-blinded placebo-controlled, single-dose, dose-escalation study of BXCL501 that enrolled 42 adult volunteers across various dosing groups. The primary endpoints of the study were PK and safety, while secondary endpoints included assessment of pharmacodynamics (PD) and the relationship between BXCL501 concentrations and PD endpoints.

Findings from the study indicate that BXCL501 rapidly achieved targeted exposure levels consistent with the levels observed in the intravenous (“IV”) Dex study in schizophrenia patients that the Company announced in November 2018. Results from the Phase 1 study also showed dose-proportional PK consistent with the IV Dex study with PD effects lasting 4 to 6 hours, which we believe are clinically favorable features.

Additionally, clinical data from the study indicates BXCL501 was well tolerated. There were no serious adverse events. The most common adverse event was drowsiness, observed at rates similar to placebo. All adverse events were mild to moderate and transient. There was no clear sedative effect in comparison with placebo. Cardiovascular changes were not clinically meaningful. A maximum tolerated dose was not reached.

Vimal Mehta, Ph.D., Chief Executive Officer of BTI, added, “We are excited to report that sublingual administration of BXCL501 in our Phase 1 study achieved drug exposures that we believe will be therapeutic in the acute treatment of agitation. Additionally, the PK profile of sublingual BXCL501 demonstrated dose proportionality and suggests the potential for a rapid onset of action. Agitation is believed to represent a multibillion dollar burden to the healthcare system severely lacking effective non-invasive treatment options. We believe that robust datasets from this new Phase 1 PK and safety study provide a strong rationale for advancing the clinical development of the BXCL501 program. With successful completion of this study and positive findings, we are now a step closer to our objective of potentially providing acutely agitated patients with a non-invasive and easy-to-administer treatment option.”

Robert Risinger, M.D., Vice President, Clinical Development of BTI, commented, “We are pleased to advance our potent selective alpha-2 agonist, BXCL501, by expanding our clinical development program. Multiple proof-of-concept studies have shown that Dex produced a calming effect in illnesses where agitation is a common symptom. If approved, we believe BXCL501 may offer patients and physicians a novel approach to treat acute agitation associated with a broad spectrum of conditions. We believe that BXCL501 may provide rapid relief in a delivery form that can easily be administered in all clinical settings where agitated patients are encountered.”

**Conference Call:**

BTI will host a conference call today at 8:30 a.m. ET. To access the call please dial 1-800-289-0438 (domestic) and 1-323-994-2082 (international) and provide the passcode 2971079. A live webcast of the call and a copy of the Company’s presentation materials that will be discussed on the call will be available on the Investors sections of the BTI website at www.bioxceltherapeutics.com. The archived webcast will be available through June 20, 2019.

**About BXCL501:**

BXCL501 is a potential first- in- class, proprietary sublingual thin film of dexmedetomidine, a selective alpha-2a receptor agonist for the treatment of acute agitation. BTI believes that BXCL501 directly targets a causal agitation mechanism and using IV (intravenous) Dex has shown anti-agitation effects in multiple clinical studies.

**About BioXcel Therapeutics, Inc.:**

BioXcel Therapeutics, Inc. is a clinical stage biopharmaceutical company focused on drug development that utilizes novel artificial intelligence approaches to identify and advance next wave of medicines in neuroscience and immuno-oncology. BTI’s drug re-innovation approach leverages existing approved drugs and/or clinically validated product candidates together with big data and proprietary machine learning algorithms to identify new therapeutic indices. BTI’s two most advanced clinical development programs are BXCL501, a sublingual thin film formulation designed for acute treatment of agitation resulting from neuropsychiatric disorders, and BXCL701, an orally administered systemic innate immunity activator designed for treatment of a rare form of prostate cancer and for treatment of pancreatic cancer in combination with other immuno-oncology agents. For more information, please visit www.bioxceltherapeutics.com.

**Forward-Looking Statements**

This press release includes “forward-looking statements” within the meaning of the Private Securities Litigation Reform Act of 1995. Forward-looking statements in this press release 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, the planned timing of BTI’s 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; 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; 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 press release. Any such forward-looking statements represent management’s estimates as of the date of this press release. 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 press release.

**Contact Information:**

BioXcel Therapeutics
The Ruth Group
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646-536-7026
jmohite@theruthgroup.com
Source: BioXcel Therapeutics, Inc.

Exhibit 99.2


bioxcel
therapeutics
(NASDAQ: BTAI)
BXCL501: Top Line Results from Phase 1 Pharmacokinetic and Safety Study in Healthy Volunteers
BioXcel Therapeutics, 555 Long Wharf Drive, New Haven, CT 06511 | www.bioxceltherapeutics.com

## 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, the planned timing of 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; 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; 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.

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# Agenda

*BXCL501 Program: Acute Treatment of Agitation*

## Program Overview

## Top Line Results from Healthy Volunteer Study

- Pharmacokinetics (PK)
  - Targeted Exposures
- Tolerability

## Overview of Registration Trial Path: Schizophrenia and Bipolar Disorder

- Integrated Development Plan

**Attendees**

- ✓ **Vimal Mehta,** *CEO & Founder*
- ✓ **Vincent O'Neill,** *CMO & SVP*
- ✓ **Frank Yocca,** *CSO & SVP*
- ✓ **Chetan Lathia,** *SVP & Head, Translational Medicine, Clinical Pharmacology & Regulatory Affairs*
- ✓ **Robert Risinger,** *VP, Clinical Development*

# BXCL501: Sublingual Thin Film Dexmedetomidine (Dex) for Acute Treatment of Agitation

*Agitation: A Growing Global Healthcare Issue ($40B+)*

**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*

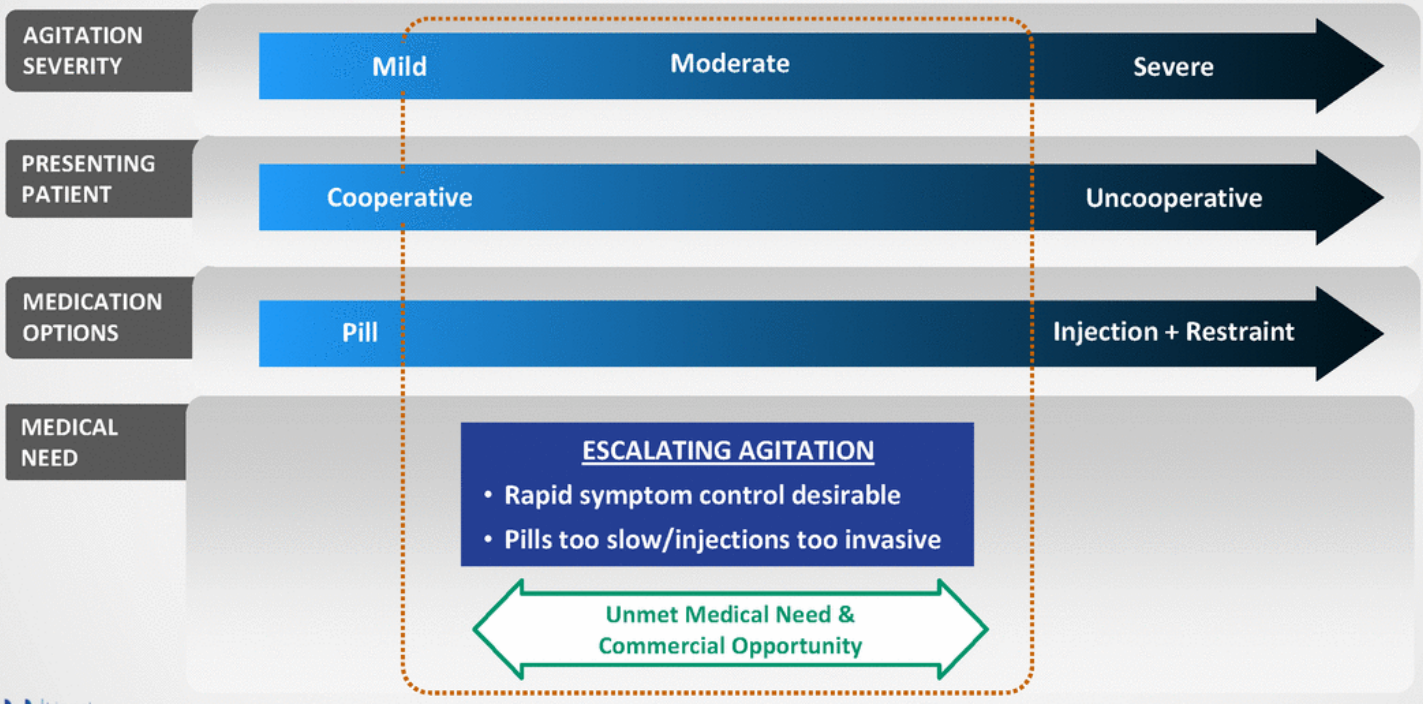
**BXCL501**: An **innovative approach:**

- ✓ Novel mechanism of action (MoA) targets a **causal agitation pathway**
- ✓ Non-Invasive, easy to administer **sublingual film** with **rapid onset of action**

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

## Treatment Across the Agitation Continuum

*Targeting Moderate Agitation Key To Improving Care*



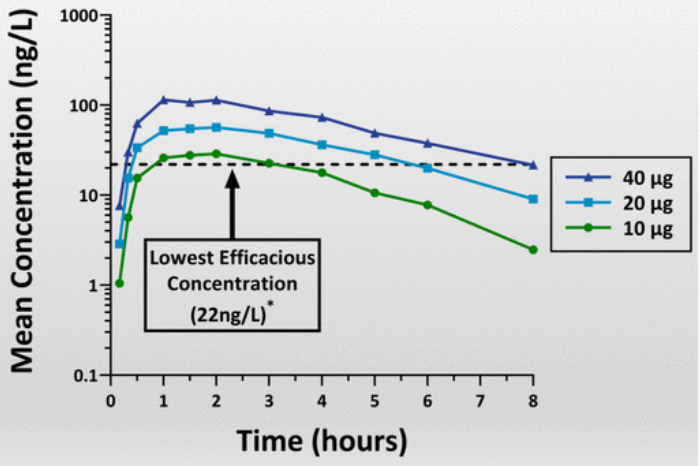
# Top Line Results From Healthy Volunteer Study

## Characterize Exposure Levels and Define Therapeutic Doses

- Double blind, placebo-controlled, single ascending dose, PK study
  - Healthy adult volunteers ages 18-65 (N = 42, 20 female)
  - Single center study
  - 3 Doses: 10, 20, 40 µg
- Primary objective:
  - Determine PK, safety and tolerability of various film strengths

# BXCL501 Rapidly Achieved Targeted Exposures

*BXCL501 Exhibited Predictable PK*




* Estimated concentration level based on Company observations in prior IV Dex study.

- **Rapidly delivered targeted exposures**
  - Consistent with therapeutic responses seen in the IV Dex schizophrenia study
- **Predictable and dose proportional PK**
  - Enables dose selection for future development
- **Pharmacodynamic (PD) effects lasted 4-6 hours**
  - Optimal treatment duration

## BXCL501 was Well Tolerated

*Consistent Safety Profile Between Sublingual Film And IV Dex*

### Tolerability Observed Across Broad Range Of Doses

- No serious adverse events (AEs)
- All AEs were Grade 2 or below (mild to moderate) and transient
  - Most common AE was drowsiness, observed at rates similar to placebo
  - Cardiovascular changes were not clinically meaningful
- No clear sedative effect for treatment group vs. placebo
- Maximum tolerated dose was not reached

# Positive Human Proof of Concept: Acute Reduction of Agitation in 4 Indications

*Safety Profile And Exposure Levels Were Consistent Across Indications With IV Dex*

## SCHIZOPHRENIA

**90% Response**

- 14 patient study (10 treatment + 4 placebo)
- PEC/RASS scores indicate de-agitation without excessive sedation

**PEC = Positive and Negative Symptom Scale-Excitatory Component*

## DEMENTIA

**70% Response**

- 14 patient study (10 treatment + 4 placebo)
- RASS* score of -1

**RASS = Richmond Agitation Sedation Scale*

**105 Subject Experience**

## DELIRIUM

**100% Response**

- 132 patients (46 refractory to haloperidol)
- **46/46 responded to IV Dex** in reducing agitation

*Carrasco et.al., Critical Care Medicine: July 2016, Vol 44, Issue 7, pp. 1295-1309*

## OPIOID WITHDRAWAL

**100% Response**

- 15 subject study (10 treatment + 5 placebo)
- 50% reduction in COWS total score

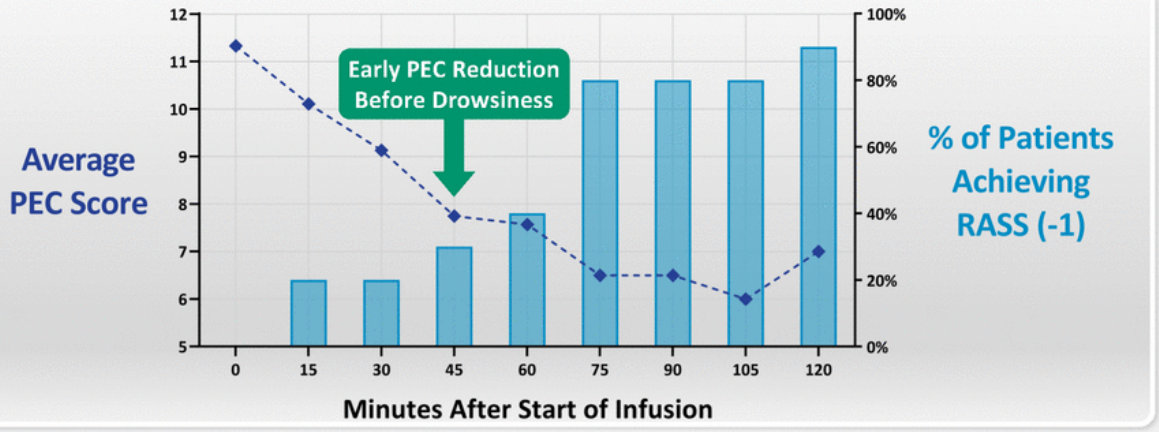
**COWS = Clinical Opiate Withdrawal Scale*

# Human Proof of Concept: IV Dex Reduced Agitation in Schizophrenia Patients

*Translating Efficacious Exposures From IV Dex To Sublingual Film*

**Study Design**

- Randomized, placebo-controlled dose-ranging study
- 14 patients [10 treatment + 4 placebo]
- Primary endpoint: RASS of -1
- Secondary endpoint: PEC score of 7 or below



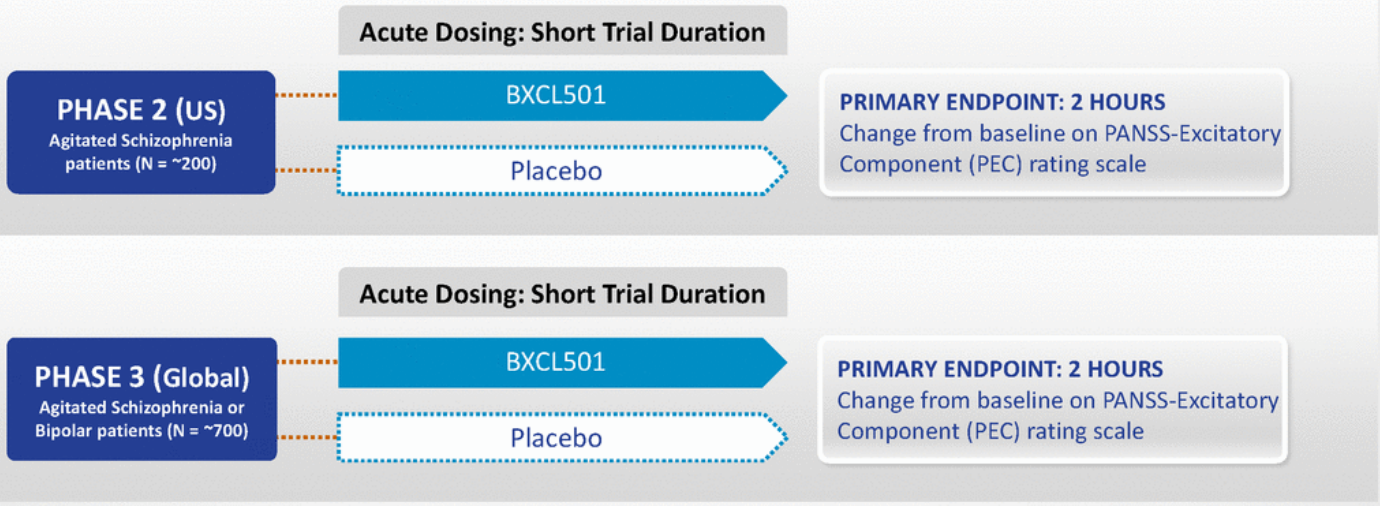

**BXCL501 PK Study Exposures Consistent with Reduction in PEC Scores**

# Overview of Registration Trial Path: Schizophrenia and Bipolar Disorder

# BXCL501 Integrated Clinical Development Plan

*Acute Agitation Studies: Short Duration With Easily Measurable Clinical Endpoints*

## Randomized, Double-blind, Placebo-controlled Multi-center Studies



## Key Targeted Milestones for Value Creation

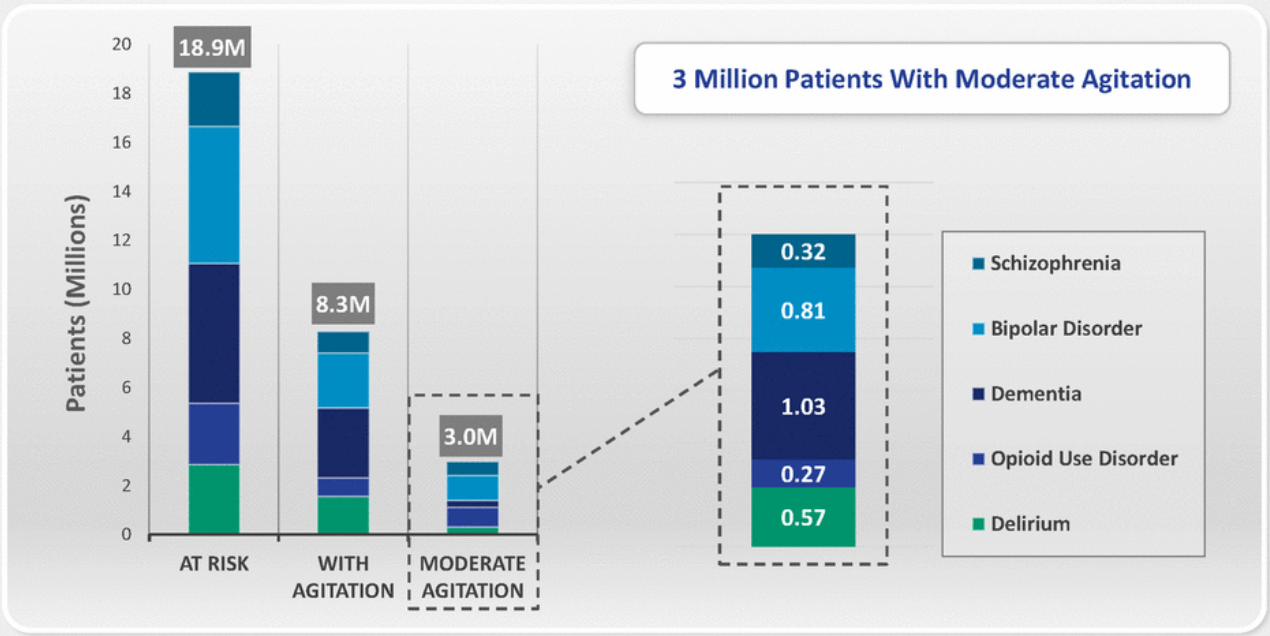
*On Track For First NDA Submission In 2H 2020*


| BXCL501 Anticipated Timeline (Schizophrenia / Bipolar Disorder) | Phase 2 Trial Initiation | ★ Phase 2 Data Readout | Phase 3 Trial Initiation | ★ Phase 3 Data Readout | ★ First NDA Submission |
|---|---|---|---|---|---|

**Development plans for Agitated Dementia, Opioid Withdrawal and Delirium will be presented through 2019**

# BXCL501 US Commercial Opportunity

*Target Patient Population Estimated at 3 Million*




**Sources:** -Internal Company Estimates
-https://www.sccm.org/Communications/Critical-Care-Statistics
-https://psychiatryonline.org/doi/pdf/10.1176/appi.books.9780890426807
-https://www.samhsa.gov/data/
-https://www.nimh.nih.gov/health/statistics/index.shtml

# Please Join Us For Our Investor Day On May 22nd

## See Agenda In Company Press Release Issued On May 13th

**Dr. Vimal Mehta,** CEO

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