

**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)  
**June 7, 2018**

**BioXcel Therapeutics, Inc.**

(Exact name of registrant as specified in its charter)

**Delaware**

(State or other jurisdiction of  
incorporation)

**001-38410**

(Commission File Number)

**82-1386754**

(I. R. S. Employer  
Identification No.)

**780 East Main Street  
Branford, CT 06405**

(Address of principal executive offices, including ZIP code)

**(203) 643-8060**

(Registrant's telephone number, including area code)

**Not Applicable**

(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:

- Written communications pursuant to Rule 425 under the Securities Act (17 CFR 230.425)
- Soliciting material pursuant to Rule 14a-12 under the Exchange Act (17 CFR 240.14a-12)
- Pre-commencement communications pursuant to Rule 14d-2(b) under the Exchange Act (17 CFR 240.14d-2(b))
- Pre-commencement communications pursuant to Rule 13e-4(c) under the Exchange Act (17 CFR 240.13e-4(c))

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

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.

**Item 7.01 Regulation FD Disclosure.**

BioXcel Therapeutics, Inc. (the "Company") has prepared presentation materials (the "Presentation Materials") that management intends to use from time to time on and after June 7, 2018, in presentations about the Company's operations and performance, including a presentation at the Jefferies Global Healthcare Conference being held in New York, New York on June 5 - 8, 2018. The Presentation Materials are furnished as Exhibit 99.1 to this Current Report on Form 8-K.

The information contained in the Presentation Materials is summary information that should be considered within the context of the Company's filings with the Securities and Exchange Commission and other public announcements that the Company may make by press release or otherwise from time

to time. The Presentation Materials speak as of the date of this Current Report on Form 8-K. While the Company may elect to update the Presentation Materials in the future or reflect events and circumstances occurring or existing after the date of this Current Report on Form 8-K, the Company specifically disclaims any obligation to do so.

The information in this Item 7.01 and Exhibit 99.1 of this Current Report on Form 8-K is furnished and shall not be deemed to be “filed” for the 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. The information in this Item 7.01 and Exhibit 99.1 of this Current Report on Form 8-K shall not be incorporated by reference into any filing under the Securities Act of 1933, as amended, or the Exchange Act, whether made before or after the date of this Current Report, regardless of any general incorporation language in any such filing.

**Item 9.01 Financial Statements and Exhibits.**

(d) Exhibits.

<u>Exhibit No.</u>	<u>Description</u>
99.1	<a href="#">Investor Presentation Materials</a>

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**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: June 7, 2018

**BIOXCEL THERAPEUTICS, INC.**

/s/ Vimal Mehta, Ph.D.  
\_\_\_\_\_  
Vimal Mehta, Ph.D.  
Chief Executive Officer

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bioxcel  
therapeutics

(NASDAQ: BTAI)



Next Wave of Medicines

BioXcel Therapeutics, 780 East Main Street, Branford, CT 06405 | [www.bioxceltherapeutics.com](http://www.bioxceltherapeutics.com)

## Safe Harbor Statement

This document may contain forward-looking statements. Such forward-looking statements are characterized by future or conditional verbs such as “may,” “will,” “expect,” “intend,” “anticipate,” “believe,” “estimate” and “continue” or similar words. You should read statements that contain these words carefully because they discuss future expectations and plans, which contain projections of future results of operations or financial condition or state other forward-looking information. Such statements are only predictions and our actual results may differ materially from those anticipated in these forward-looking statements.

We believe that it is important to communicate future expectations to investors. However, there may be events in the future that we are not able to accurately predict or control. Factors that may cause such differences include, but are not limited to, the uncertainties associated with our limited operating history, product development, the regulatory approval process of the FDA, the market for our product candidates, the success of BXCL501 and BXCL701, the risks associated with dependence upon key personnel and the need for additional financing. Except as required by law, we do not assume any obligation to update forward-looking statements as circumstances change.

These forward-looking statements are based on certain assumptions and are subject to risks and uncertainties, including those described in the “Risk Factors” section and elsewhere in the Company’s filings with the U.S. Securities and Exchange Commission, which are available at [www.sec.gov](http://www.sec.gov) and <https://ir.bioxceltherapeutics.com/all-sec-filings>.

# First Public Company Unleashing the Power of AI Across the Entire R&D Value Chain

*Developing high value therapeutics in neuroscience and immuno-oncology*

## UNBIASED, SYSTEMATIC ANALYSIS OF THE ENTIRE DISEASE SPACE

*50+ million publications and other data sources*

## UNEARTHING NOVEL CONNECTIVITIES IN DISEASE PATHOPHYSIOLOGIES

*R&D expert infused, proprietary algorithms and development process*

## IDENTIFICATION OF TRANSFORMATIONAL TREATMENT MODALITIES

*Leveraging the pool of > 2500 phase II/III clinical candidates and approved medicines*

## OPTIMIZES R&D ECONOMICS, SHORTENS DEVELOPMENT TIMELINES AND INCREASES PROBABILITY OF SUCCESS

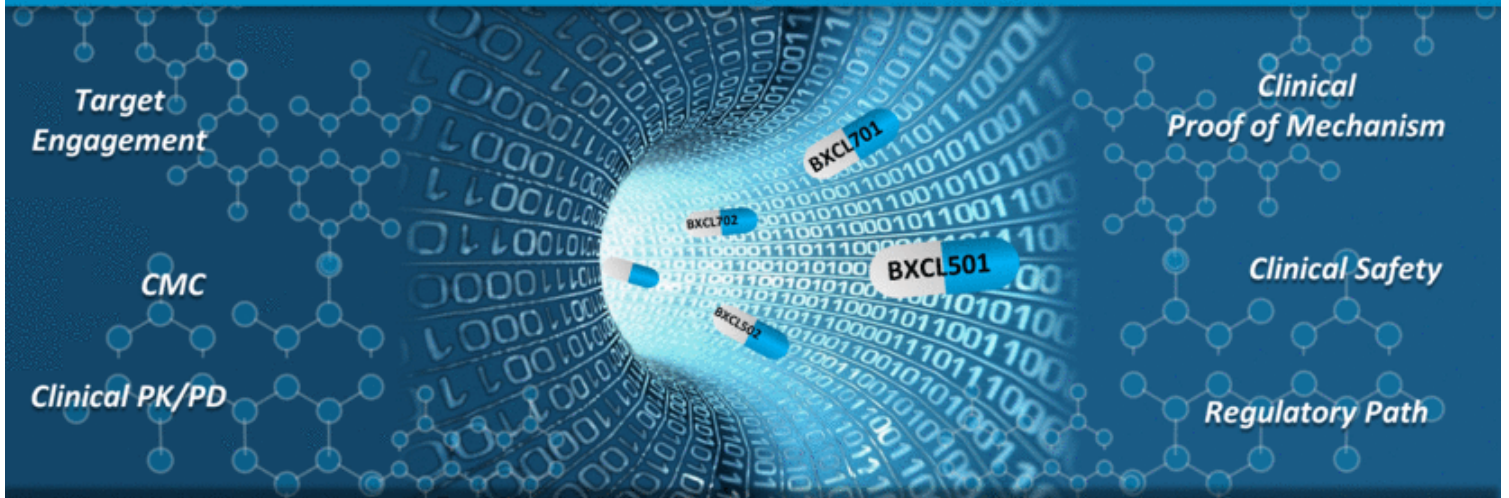
# Applying Artificial Intelligence to Drug Discovery and Development

*Through Exclusive Relationship with BioXcel (Parent)*



# Our Focus : Neuroscience and Immuno-Oncology

De-risked approach



Large Market Opportunity



Faster Path to Market



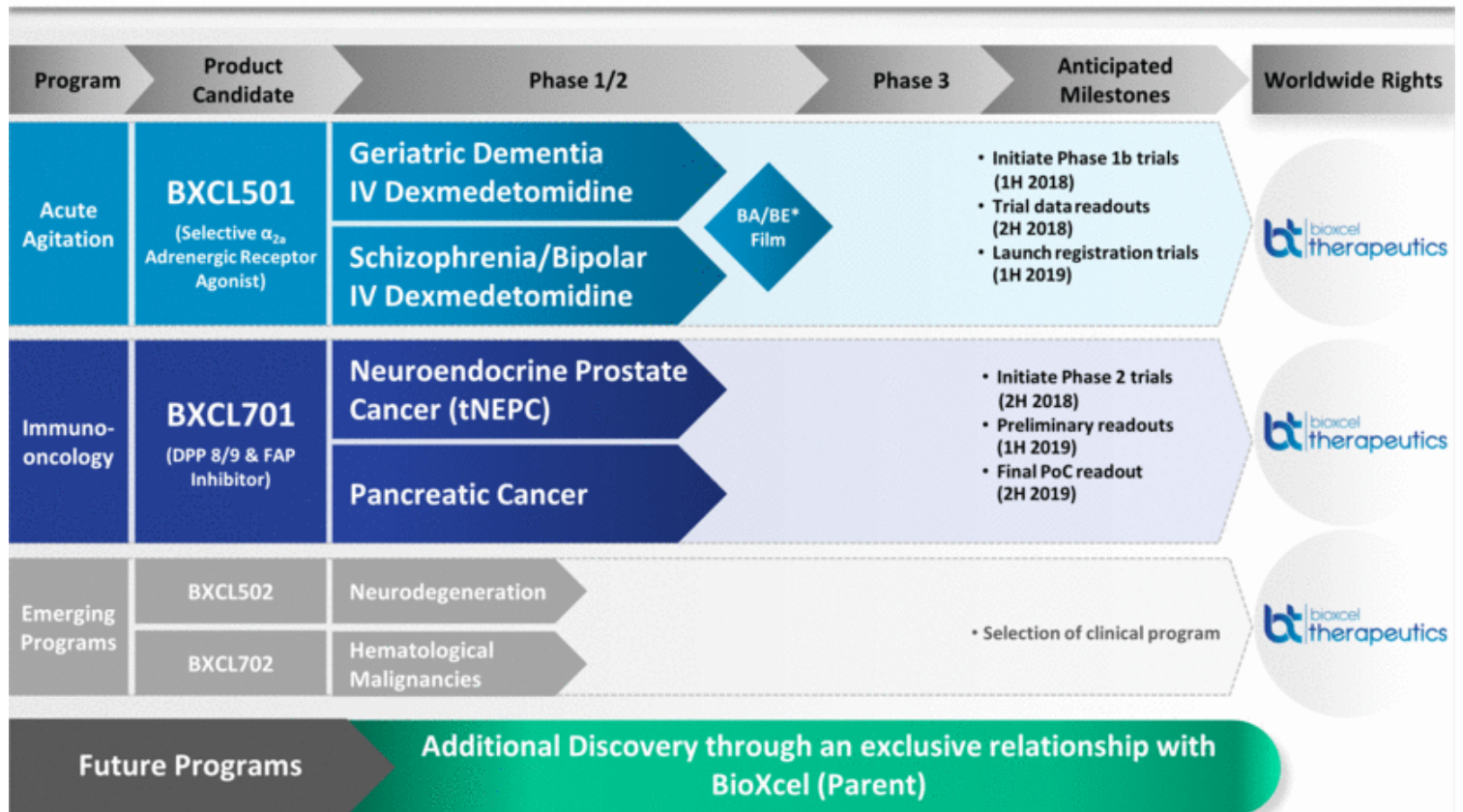
Lower Cost of Development



Higher Probability of Success

# BioXcel Therapeutics Pipeline: Rapid Human PoC and Development Path

First-in-class neuroscience and immuno-oncology pipeline with multiple near-term milestones



\*Bridging bioavailability/bioequivalence (BA/BE) study for optimizing BXCL501 sublingual thin film dose for phase 3 registration trials





## Clinical Programs

*BXCL501*



## Rapid Clinical Development and Regulatory Approval Path (505(b)(2))

Agitation resulting from Alzheimer's and Schizophrenia / Bipolar disease

- Agitation: a growing **global healthcare issue (\$40B+)**
- Existing treatments are suboptimal; invasive with severe **side effects**
- BXCL501: **innovative approach** for acute treatment of agitation
  - *Directly targets a **causal agitation mechanism***
  - ***Rapid onset of action; easy to administer sublingual film***
  - *Established **regulatory and reimbursement path (Adasuve)***



# BXCL501: The Sublingual Film Formulation of Dexmedetomidine (Dex)

Sublingual film formulation under development with multiple dose strengths

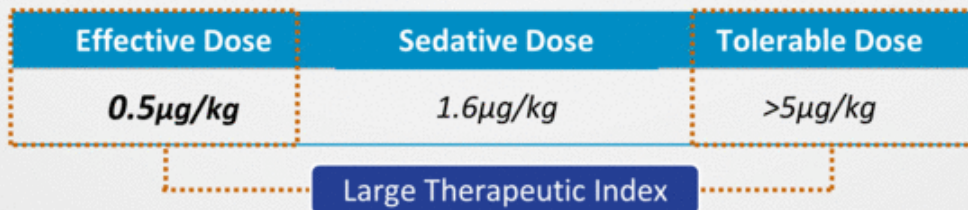
## The Right Pharmacology and Safety Profile

- Initially developed by Hospira as anesthetic/sedative; US 1999
- Prescribed to more than 8M patients
- Studied in 120 clinical trials
- Demonstrated efficacy in managing agitation from delirium in ICU



## Ideal Pharmaceutical Properties for a non-invasive Sublingual Film Formulation

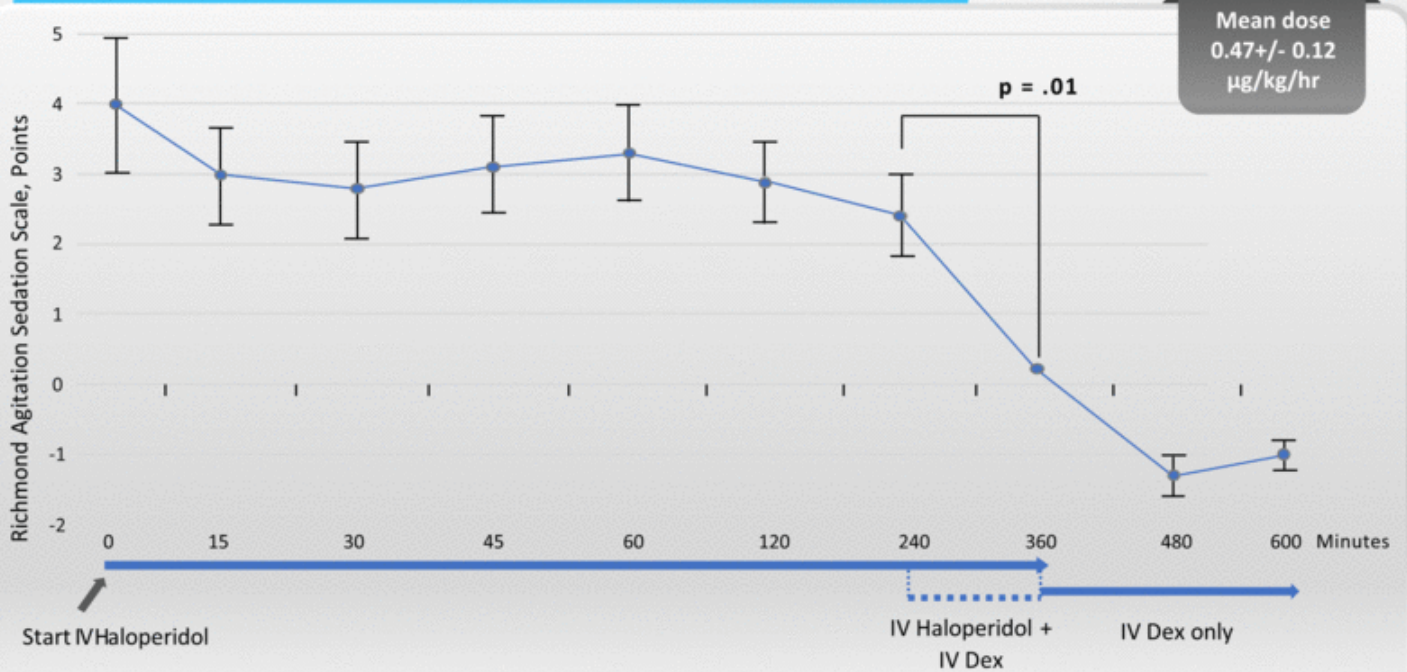
- Low dose (in mcg) ideal for film formulation
- Good aqueous solubility for rapid absorption
- Favorable chemical stability in oral mucosa
- Tasteless



# Acute Agitation Clinical Study Shows Easily Measured Endpoints

Hyperactive delirium patients refractory to haloperidol are difficult to treat

## BXCL501 MoA Proven to Treat Agitated Delirium in Elderly

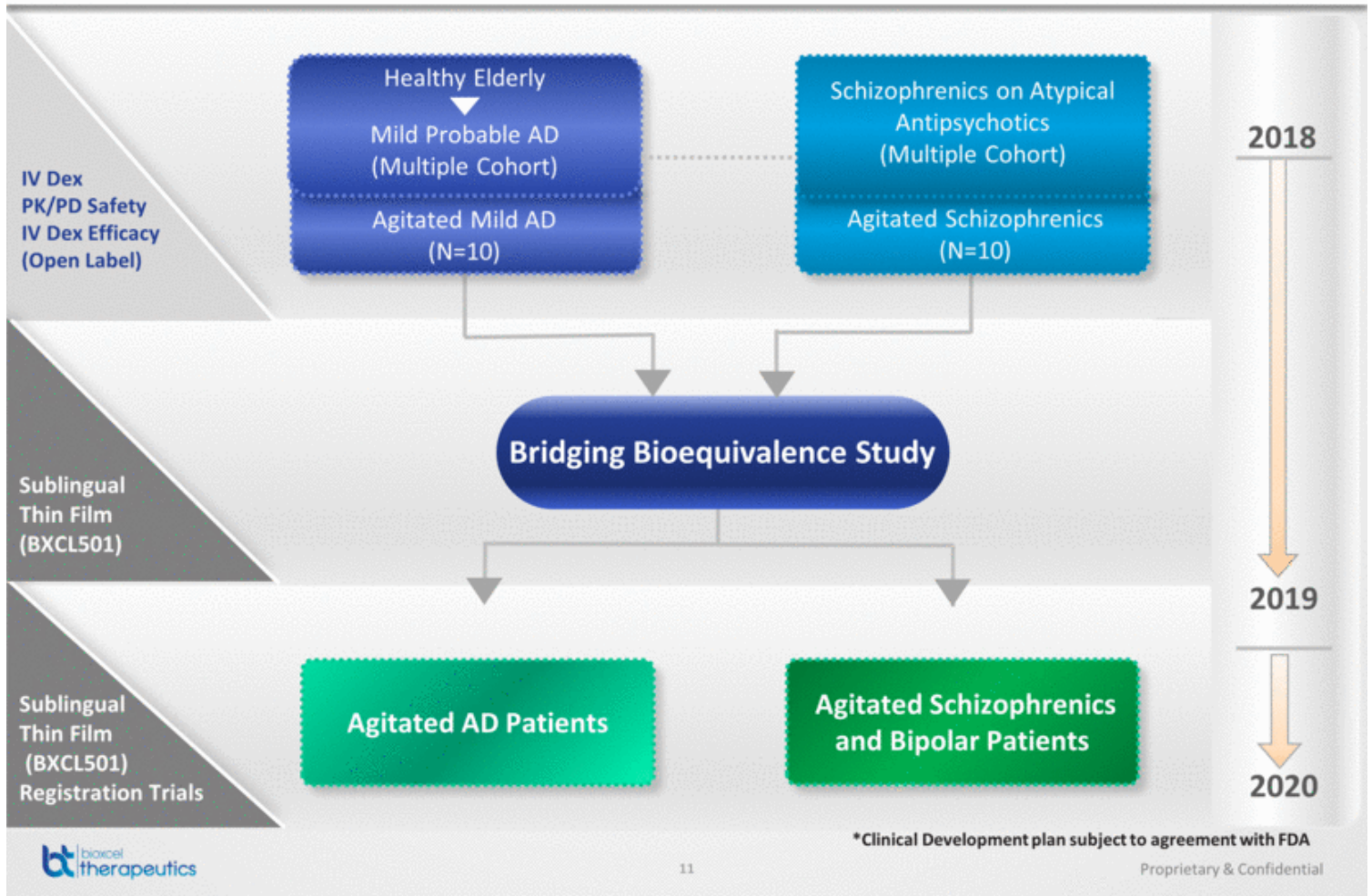


**All Haloperidol-refractory Agitated Patients (n=46) were Calmed by Dex Treatment**

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

# BXCL501 Integrated Clinical Development Plan

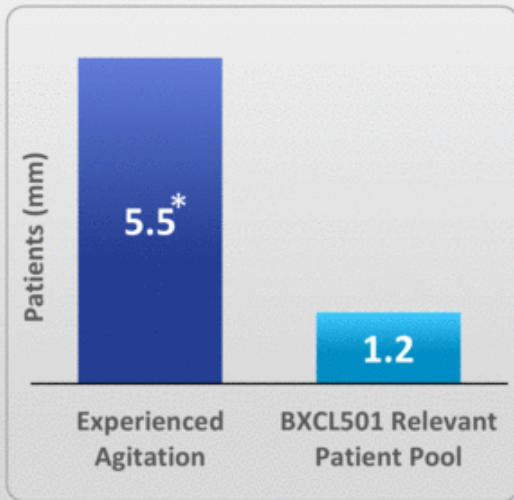
Acute agitation studies: short with easily measurable clinical endpoints



# Healthcare Costs Associated with Agitation are a Significant Economic Burden

Cost of acute agitation treatment across neuroscience disorders estimated >\$40 billion

## U.S. Addressable Market for Acute Treatment of Agitation



12 – 24 episodes per patient

Reimbursement; \$145 per episode at launch

### Indication Expansion

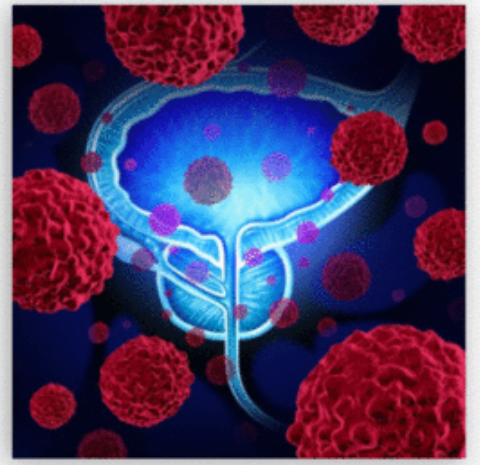
Delirium

Alcohol Withdrawal DTs

PTSD/Hyperarousal

Pre-MRI Anxiety

## BXCL501: Rapid Development Path with Large Market Potential



## Clinical Programs

*BXCL701*

## Rare Tumors with Large Market Opportunity and Limited Competition *Human PoC in Melanoma with BXCL701 established*

- BXCL701 converts **“cold” (immune resistant) tumors to “hot” (immune permissive) tumors**
- Differentiated mechanism of action inhibits **DPP 8/9 & FAP**, induces **immune activation** and blocks **immuno-evasion**
- **Clinical proof of mechanism** and tolerable safety profile from 700 patients
- Potential for **accelerated approval** and **breakthrough therapy designations**
- Offers synergistic benefit in combination with **checkpoint inhibitors and other IO therapies**



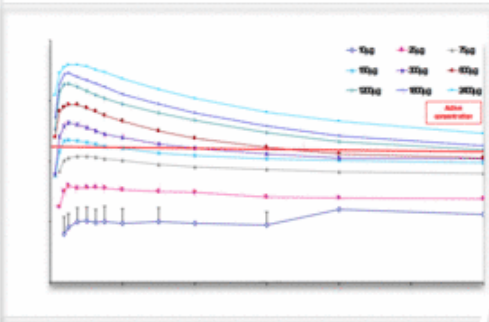
(1) <http://www.nature.com/nchembio/journal/v13/n1/abs/nchembio.2229.html?foxtrotcallback=true>



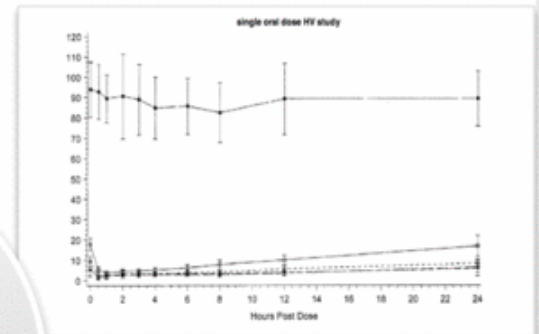
# BXCL701: Existing Clinical Evidence Enables Rapid Development Path

Data from >700 patients demonstrate well characterized human PK/Target Inhibition/PD, melanoma, and anti-tumor activity

## ✓ Dose



## ✓ Target Inhibition



### BXCL701 Human Proof of Concept

✓ Single Agent Efficacy  
~10% CR/PR with long duration, similar to Yervoy anti-CTL4

More than \$75 million investment (Point Therapeutics)



## ✓ Stimulation Of Immune Cells

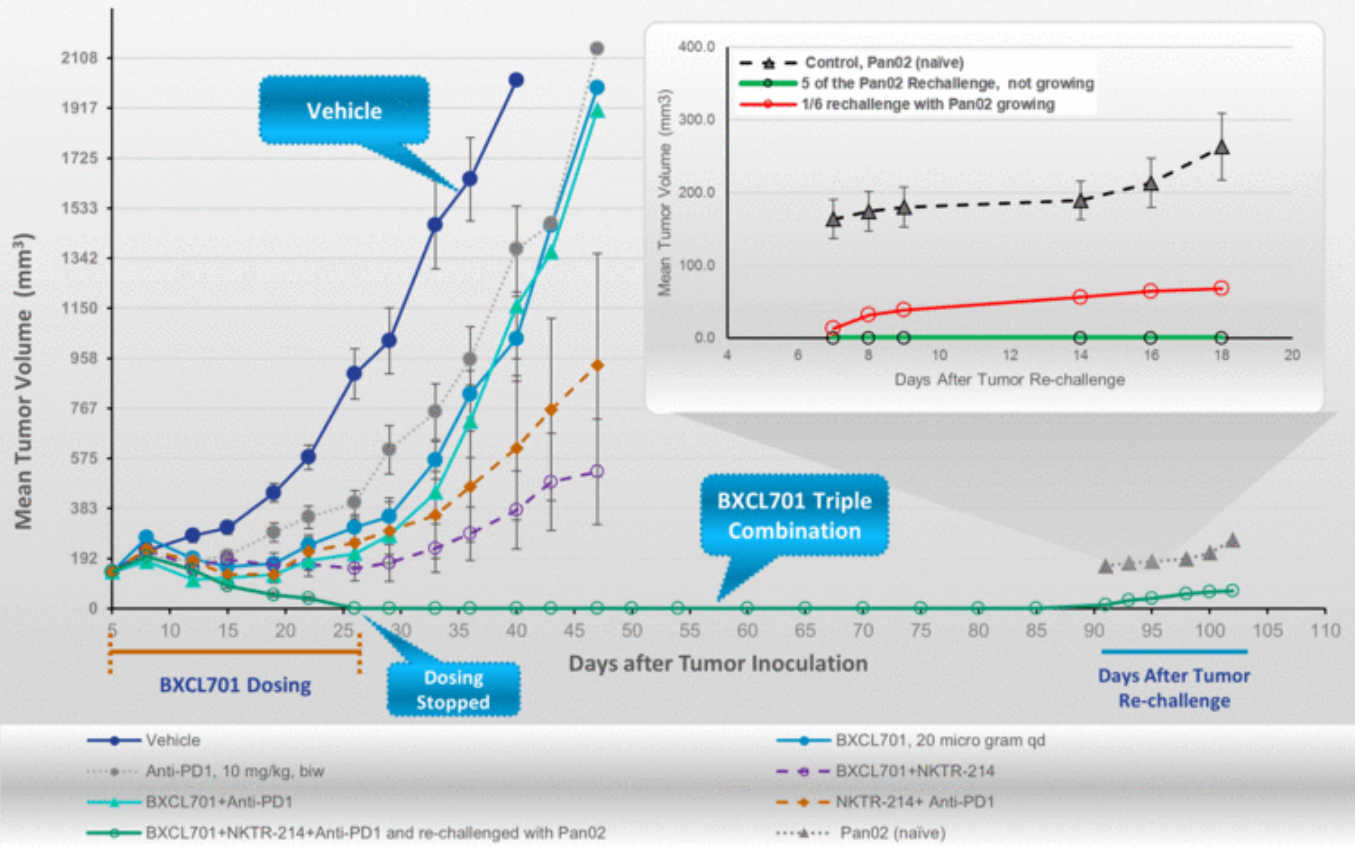
## ✓ Induction of Pro-inflammatory Cytokine

----Daily BXCL701 Dose----

Cytokine*	400mg (n=11)	600mg (n=9)	800mg (n=6)	All Telesostat (N=20)
G-CSF	7 (64)	3 (100)	5 (83)	15 (75)
IL-1α	1 (9)	0	0	1 (5)
IL-1β	5 (46)	2 (87)	5 (83)	12 (60)
IL-2	6 (55)	2 (87)	1 (17)	9 (45)
IL-6	5 (46)	2 (87)	4 (67)	11 (56)
IL-8	5 (46)	3 (100)	2 (33)	10 (50)
IL-10	4 (36)	3 (100)	5 (83)	12 (60)
TNF-α	6 (55)	2 (87)	4 (67)	12 (60)
IPN-α	2 (18)	2 (87)	1 (17)	5 (25)

# Triple Combination Achieved Complete Regression and Immunity in Pancreatic Tumors

Combo with anti-PD1 and NKTR-214 fully stimulates immune system, "curing" mice, making them resistant to new tumors



# Pancreatic Cancer Clinical Development Plan: Mechanistic and Anti-PD1 Combo Trial

*Biomarker driven development in advanced pancreatic cancer, potential breakthrough designation*

Neoadjuvant  
Proof of Mechanism Trial



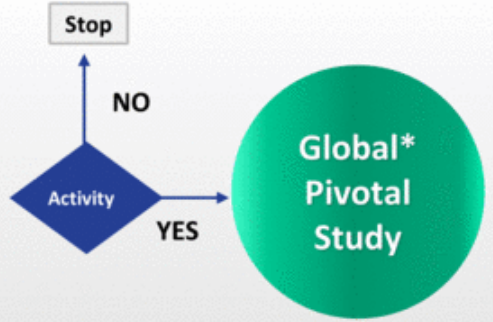
3 Weeks of BXCL701 Treatment  
Before Surgery  
Pre and Post Tissue Available  
(N=15)

Demonstration of immune  
cell infiltration/activation to  
validate MOA

Efficacy Trial in Metastatic  
Patients after First-line  
Treatment



Combo with Keytruda  
Pre and Post Biopsy  
(N=30)



**Simon 2-stage:** 15+15  
**Primary Endpoint:** ORR  
Combination: > 15%  
**Secondary Endpoint:** DoR, PFS, OS  
**Exploratory Endpoint:** Effect on immune cells (MDSC, T-cells, neutrophils)

Louis M. Weiner  
Director

Georgetown | Lombardi  
COMPREHENSIVE CANCER CENTER

# tNEPC Clinical Development Plan: Single Agent and Combination with Anti-PD1

Biomarker driven development, breakthrough and fast track designation potential

## Single Agent



**KEYTRUDA**  
(pembrolizumab)

## Combination with Keytruda



\*Expect to commence global development planning (focus on EU and Japan) during Phase 2

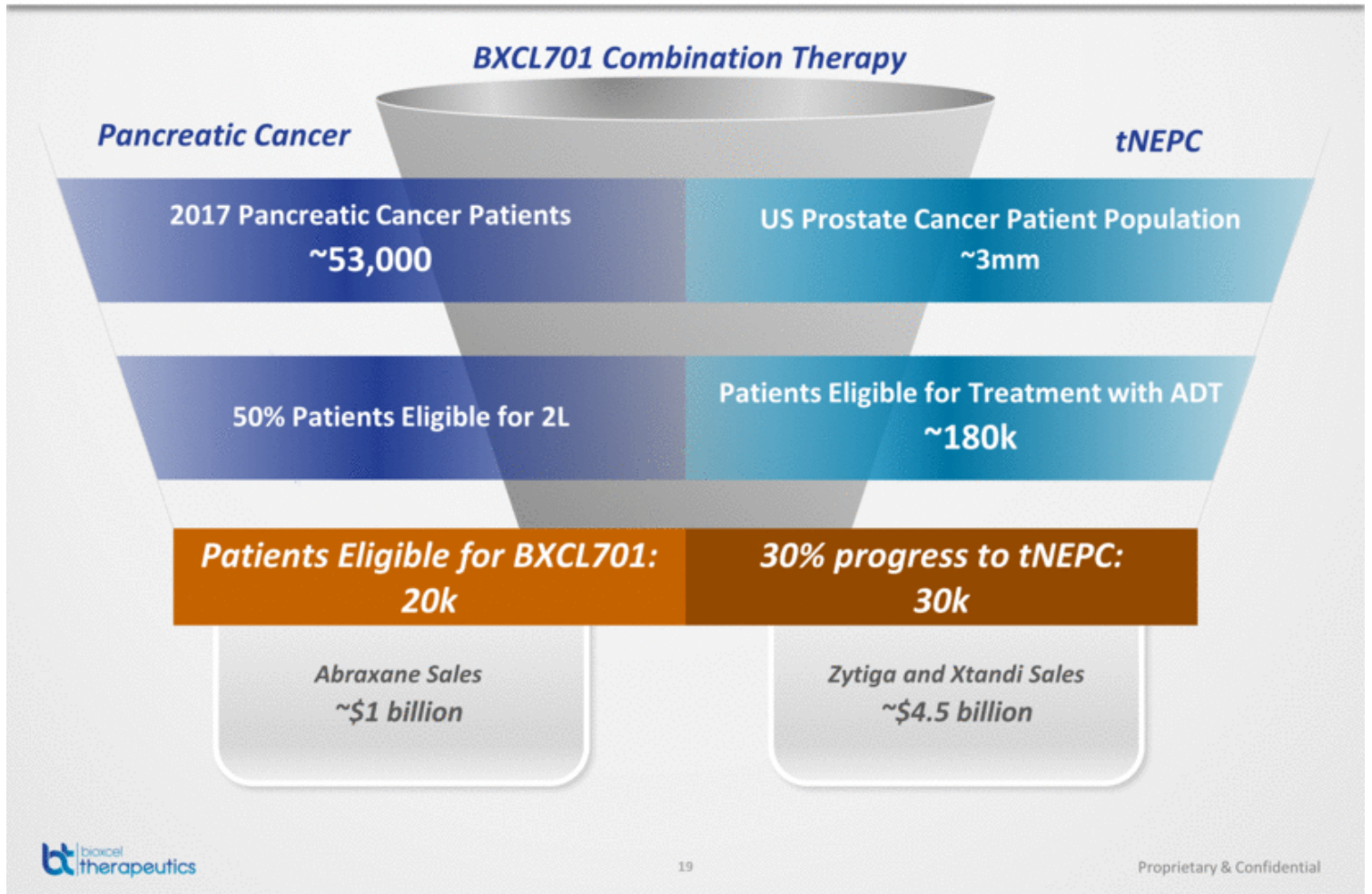
**Simon 2-stage:** 15+15  
**Primary Endpoint:** ORR  
Single agent: > 10%  
Combination: increase from ~3-5% (Keytruda single agent) to > 15%  
**Secondary Endpoint:** DoR, PFS, OS  
**Exploratory Endpoint:** Effect on immune cells (MDSC, T-cells, neutrophils)

**Emmanuel Antonarakis**  
Principal Investigator  
for Keynote 199  
Prostate Cancer



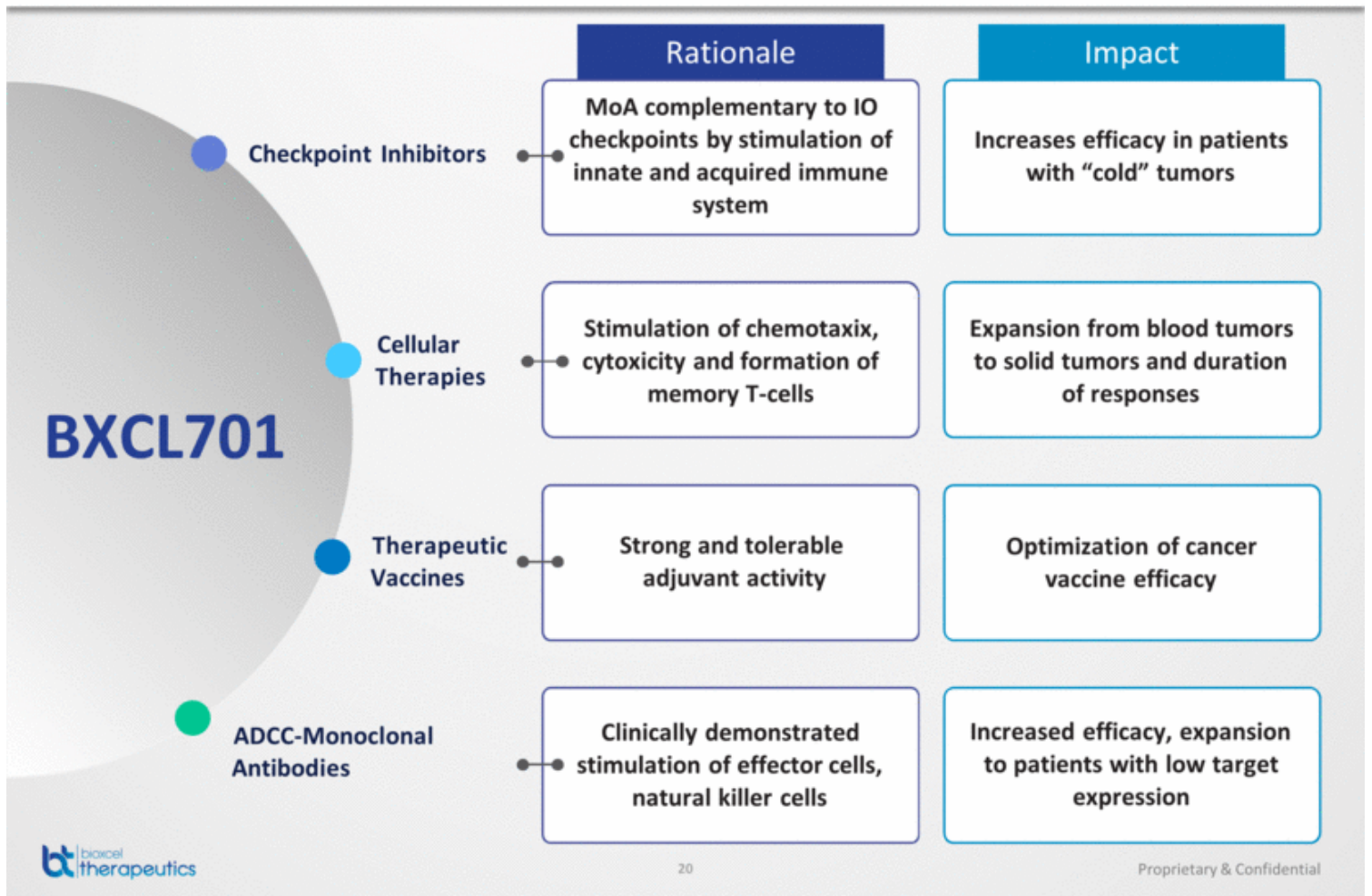
# Large Market Opportunity

Limited competition



# Offers Pipeline-in-a-Product Platform

Broad potential across multiple IO modalities





## Value Creation Catalysts

# Key Milestones for Value Creation

Two mid-stage clinical trial candidates

Drug	Indication	1H'18	2H'18	1H'19	2H'19	2020 and Beyond	
<b>BXCL501</b>	Geriatric Dementia	IV Dex Study Ongoing	<b>IV Dex Data Readout</b> PK/PD PoC Trial Bio-Equivalence Film Study Initiation	Registration Trial		NDA	
	Schizophrenia / Bipolar Disease	IV Dex Study Planned	<b>IV Dex Data Readout</b> PK/PD PoC Trial Bio-Equivalence Film Study Initiation	Registration Trial			
<b>BXCL701</b>	Neuroendocrine Prostate Cancer (tNEPC)		Single Agent & Combo Trial Initiations	<b>Preliminary Readout</b>	<b>Final PoC Readout</b>	Registration Trial	NDA
	Pancreatic Cancer (PDA)		Neoadjuvant Proof of Mechanism Trial Initiation Combination Trial Initiation	<b>Mechanistic (MOA) Readout</b>	<b>Combination Readout</b>	Registration Trial	
<b>Emerging Programs</b>	Neuroscience and Immunology		Selection of Next Candidate(s)				



### Funded to Reach Multiple Inflection Points

- Completed Initial Public Offering in March 2018, generating gross proceeds of **\$60 million**
- Major shareholders include **Fidelity (10.7%)** and **Artemis (7.7%)**
- Total cash and cash equivalents of **\$55.5 million** as of March 31, 2018

# World-Class Leadership Team Supported By Strong Board of Directors and Advisory Board

Combined experience of 150+ years in drug development with 15 approved drugs

## Management Team



**Vimal Mehta**  
CEO & Member of Board

- 25+ years experience of corporate strategy and financing
- Results-driven serial healthcare entrepreneur, investor and advisor



**Frank Yocca**  
Chief Scientific Officer

- 30+ years of pharma and biotech experience
- VP and Head, AZ; Neuroscience
- Executive Director, BMS



**Vincent J. O'Neill**  
Chief Medical Officer

- 15+ years of therapeutic experience
- CMO, Mirna Therapeutics
- VP, Sanofi-Aventis
- Group Director, Genentech
- Clinical Director, GSK



**Richard I. Steinhart**  
Chief Financial Officer

- 25+ years of corporate finance experience
- CFO, Remedy Pharmaceuticals
- SVP & CFO, MELA Sciences

Inpharmatica

CuraGen

AstraZeneca

BMS

Mirna

Sanofi-Aventis

Remedy Pharmaceuticals

MELA Sciences

## Board of Directors



**Peter Mueller**  
Chairman of Board

- 30+years pharma & biotech experience
- EVP, Global R&D/CSO Vertex
- SVP, R&D Boehringer Ingelheim
- President, R&D/CSO Axcella Health



**Steve Laumas**  
Member of Board

- 20+ years of experience in healthcare investments
- CEO, Bearing Circle Capital
- Managing Director, North Sound Capital



**Krishnan Nandabalan**  
Member of Board

- Innovator of AI Platform
- Drug discovery & development and global business development & licensing (Viibryd)



**Steven Paul**  
President & CEO, Voyager Therapeutics

- 35+ years of Neuroscience expertise
- Venture Partner, Third Rock Ventures
- CEO, Voyager Therapeutics
- Co-Founder, SAGE Therapeutics
- President, Lilly Research Laboratories



**Sheila Gujrathi**  
Member of Board,  
Five Prime Therapeutics

- 15+ years clinical development experience
- CMO, Receptos
- VP, Immunology, Bristol-Myers Squibb
- Franchise Leader, Genentech

Vertex Boehringer Ingelheim Axcella

Goldman Sachs

Genaissance

CuraGen

Eli Lilly SAGE Therapeutics Alnylam

Receptos BMS Genentech



# BioXcel Therapeutics Investment Highlights

Developing high value therapeutics in neuroscience and immuno-oncology utilizing a novel artificial intelligence platform





**Dr. Vimal Mehta, CEO**

BioXcel Therapeutics, Branford, CT 06405 USA

[vmehta@bioxceltherapeutics.com](mailto:vmehta@bioxceltherapeutics.com)