bioxcel therapeutics

(NASDAQ: BTAI)

Next Wave of Medicines

December 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, anticipated milestones, clinical development plans, the availability and results of data from clinical trials, 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.

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BioXcel Therapeutics Investment Highlights

Neuro Symptoms Based Approach

BXCL501
Sublingual Thin Film for Acute Treatment of Agitation

- Phase 3 Pivotal Trial Initiation Expected
  - 4Q 2019
- Phase 3 Data Readout
  - mid-2020

Innate Immunity Based Approach

BXCL701
Targeting Rare Cancers

- Phase 1b/2 double combination trial in Neuroendocrine Prostate Cancer (tNEPC) ongoing
- Phase 1b/2 triple combination trial in pancreatic cancer expected 2020

Triple Combination Partners

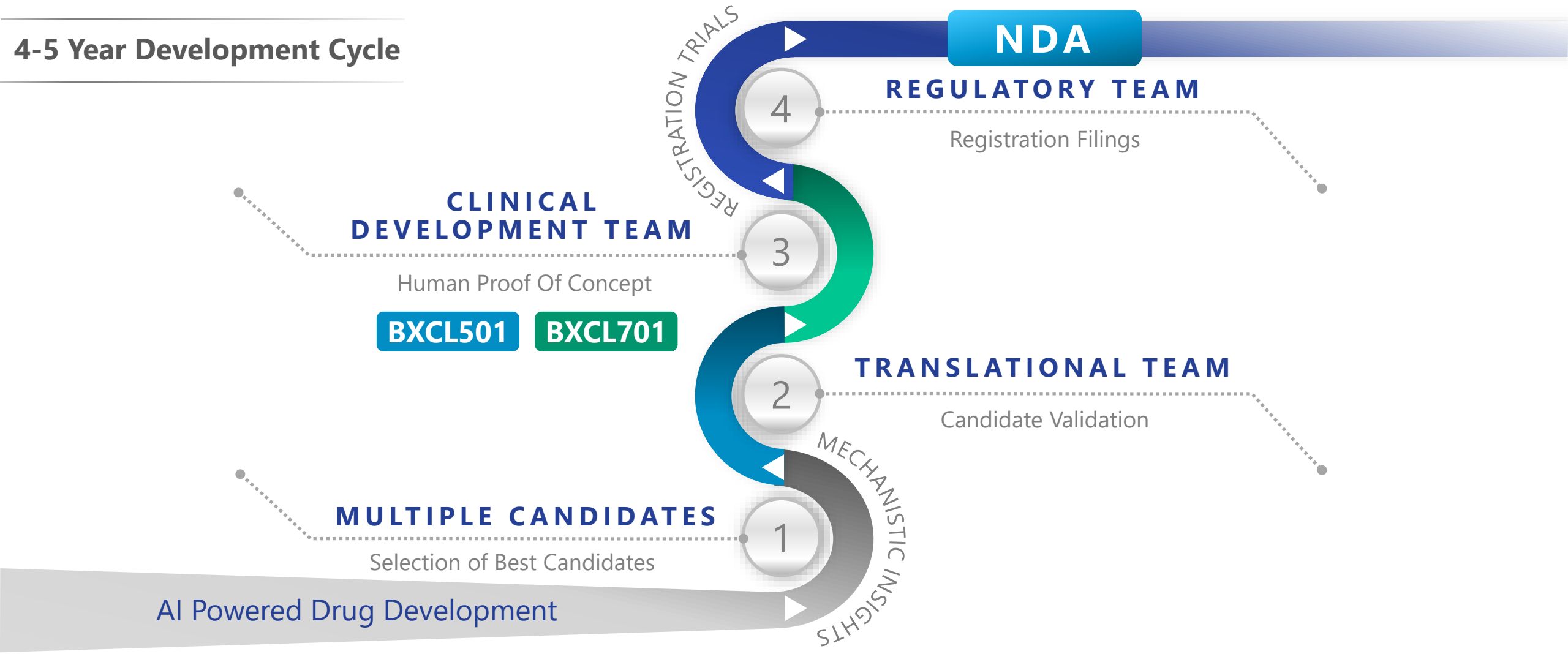
Pfizer | MERCK | NEKTAR

AI-powered Drug Development

- Improves R&D Economics
- Increases Development Efficiency
- Maximizes Probability of Success

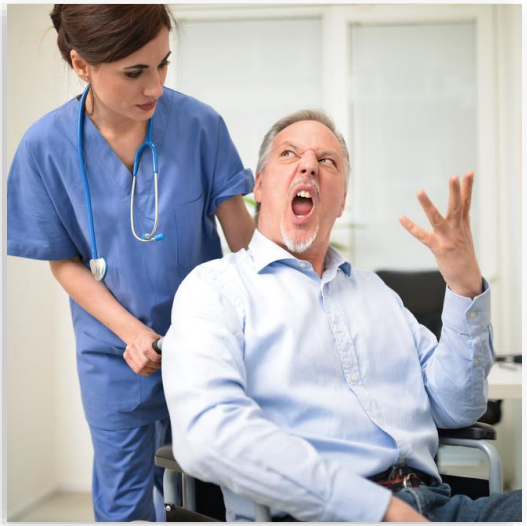
Unleashing the Power of AI Across the Entire R&D Value Chain

4-5 Year Development Cycle



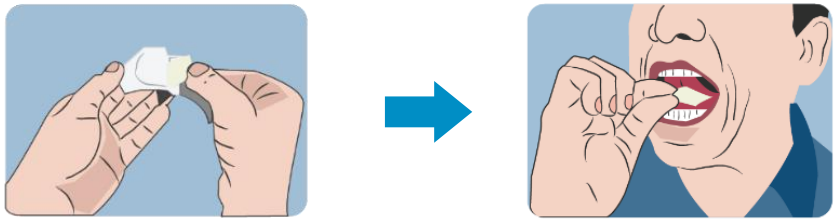
Pipeline To Rapid Human PoC and Development Path

Program	Product Candidate	Phase 1/2	Phase 2/3	Anticipated Milestones	Worldwide Rights
Treatment of Acute Agitation	BXCL501 (Selective α_{2a} Adrenergic Receptor Agonist)	Phase 1b Completed → Schizophrenia/Bipolar		• Phase 3 schizo/bipolar – Trial initiation (4Q 2019) – Data readout (mid-2020) – NDA submission (2H 2020) • Alzheimer's/Dementia trial initiation (2H 2019)	bioxcel therapeutics
		Phase 1b/2 Trial Initiating → Alzheimer's/Dementia			
Immuno-Oncology	BXCL701 (DPP 8/9 & FAP Inhibitor)	Neuroendocrine Prostate Cancer (tNEPC) (double combination)		• tNEPC data readouts (2H 2019 and 1H 2020) • Pancreatic data readouts (2020)	bioxcel therapeutics
		Pancreatic Cancer (triple combination)			
Pipeline Expansion	BXCL501	Opioid Withdrawal, Delirium		• New indications and geography expansion	bioxcel therapeutics
	BXCL701	Exploring Multiple Tumor Types			
Future Programs		Additional Discovery Through an Exclusive AI Relationship with BioXcel Corporation (parent)			



Clinical Programs

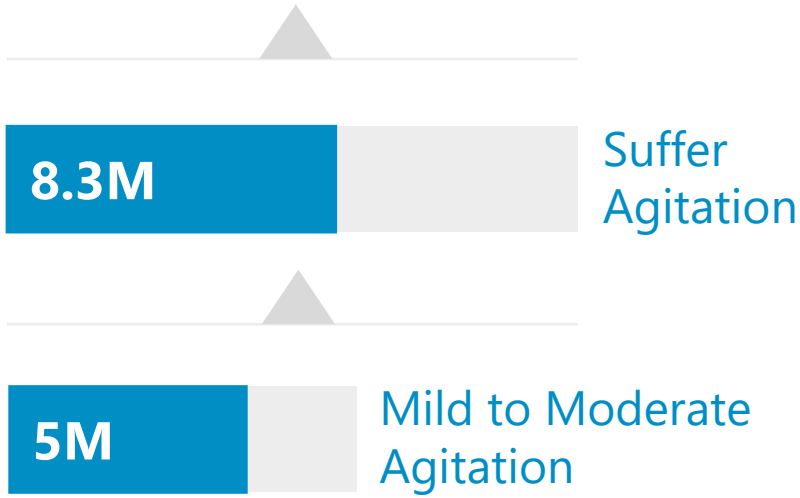
BXCL501: Potential First in Class Sublingual Thin Film Dexmedetomidine (Dex) for Acute Treatment of Agitation



Agitation is an Unmet Medical Need

- Common and expensive phenomenon associated with multiple psychiatric conditions
  - 8.3 million suffer each year in the US
  - $40 billion per year health care burden
- Rapid symptom relief with a non-invasive approach is desired
  - Patients experience multiple episodes per year

~19M At Risk



Proprietary Sublingual Thin Film of Dex* for Acute Treatment of Agitation

Treatment Options

Current Treatments are Suboptimal:

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

FDA

Fast Track Designation

BXCL501: Novel Mechanism of Action (MoA)

- Selective alpha-2a receptor agonist designed to directly target causal agitation mechanism

*Dexmedetomidine

Proprietary Formulation and Scale Up Manufacturing

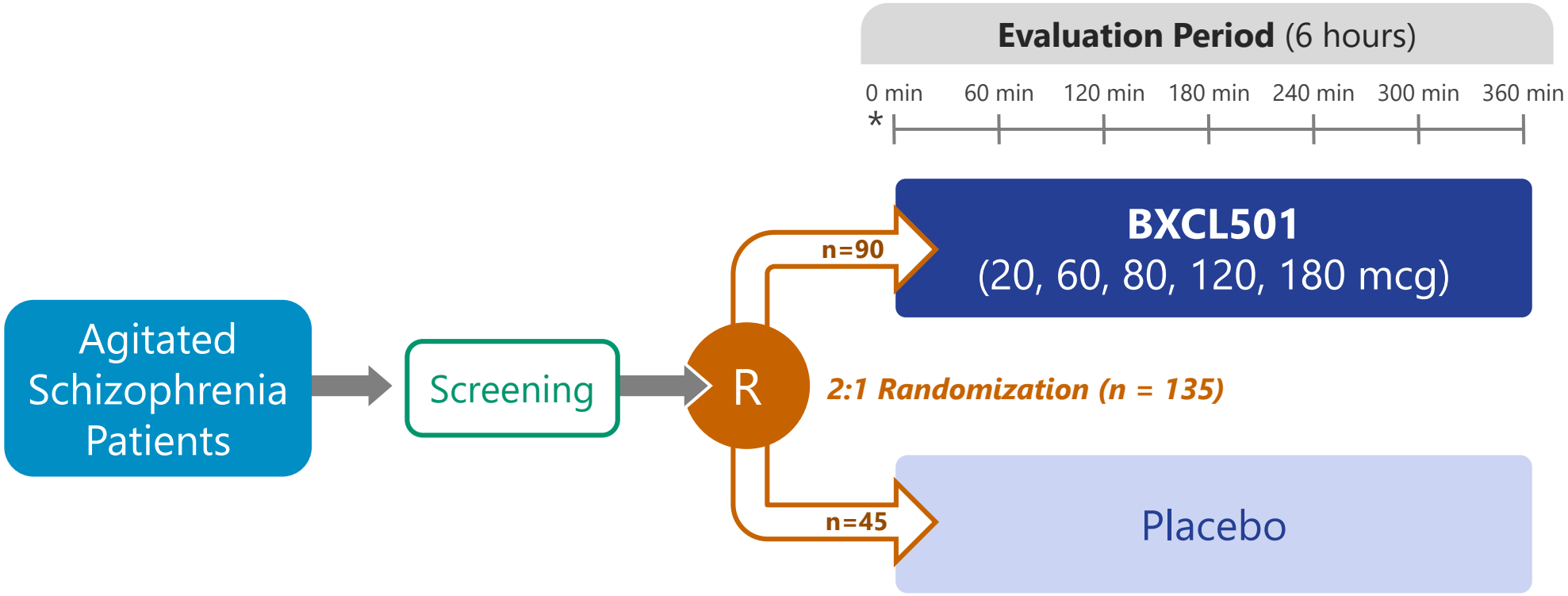
118 subjects dosed: Healthy volunteers (28) and Agitated Schizophrenia patients (90)

- Transitioned to Registrational Drug Product Process
  - Manufacturing phase 3 / registrational batches
  - Commercial scale-up planned for product launch

- Proprietary, Immediate Delivery, Sublingual Thin Film Product
  - Muco-adhesion properties designed for optimizing compliance
  - Adaptable technology enables broad dose range
  - Flexible for potential combination of multiple drugs on a single film

Phase 1b Clinical Trial in Agitated Schizophrenia Patients

Assessing Agitation Episodes in Schizophrenia



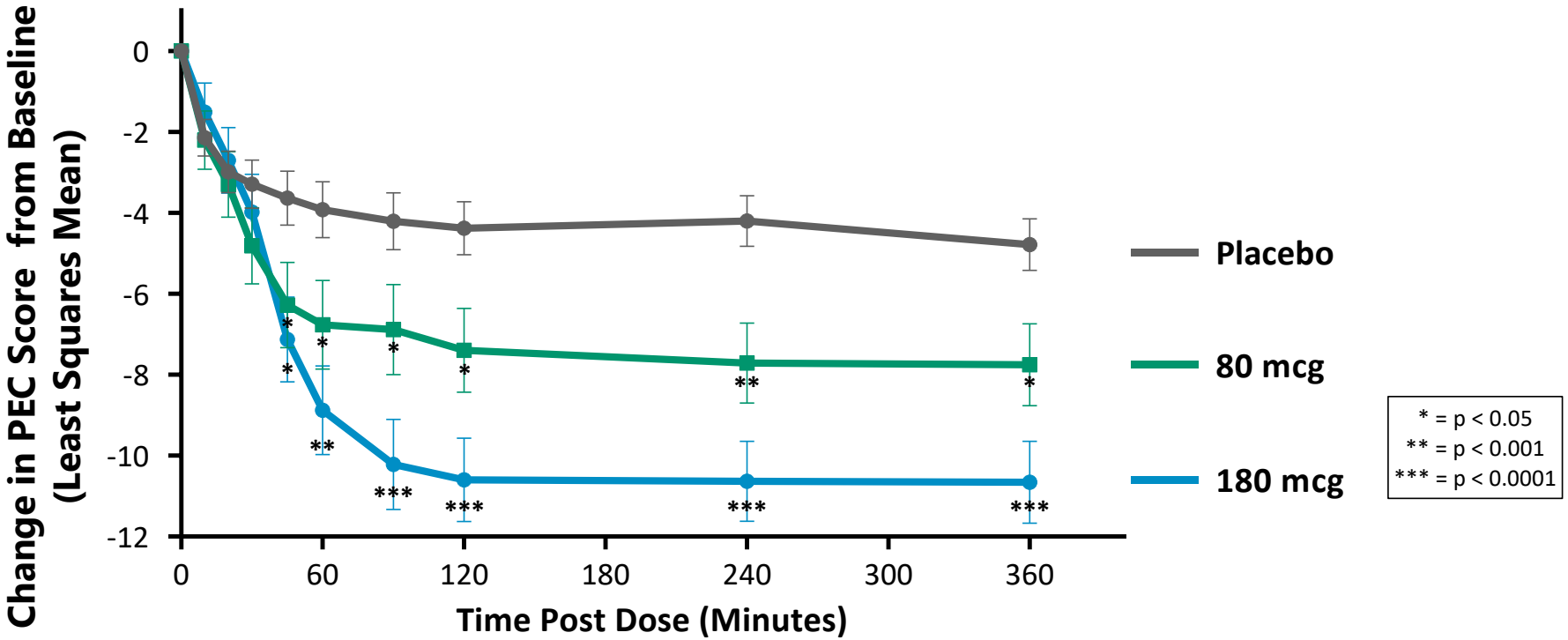
✓ Initiated **May 2019** →
✓ Completed **July 2019**

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

* Patients Dosed

Primary Endpoint: Change in PEC Score from Baseline

Clinically meaningful, rapid and durable responses



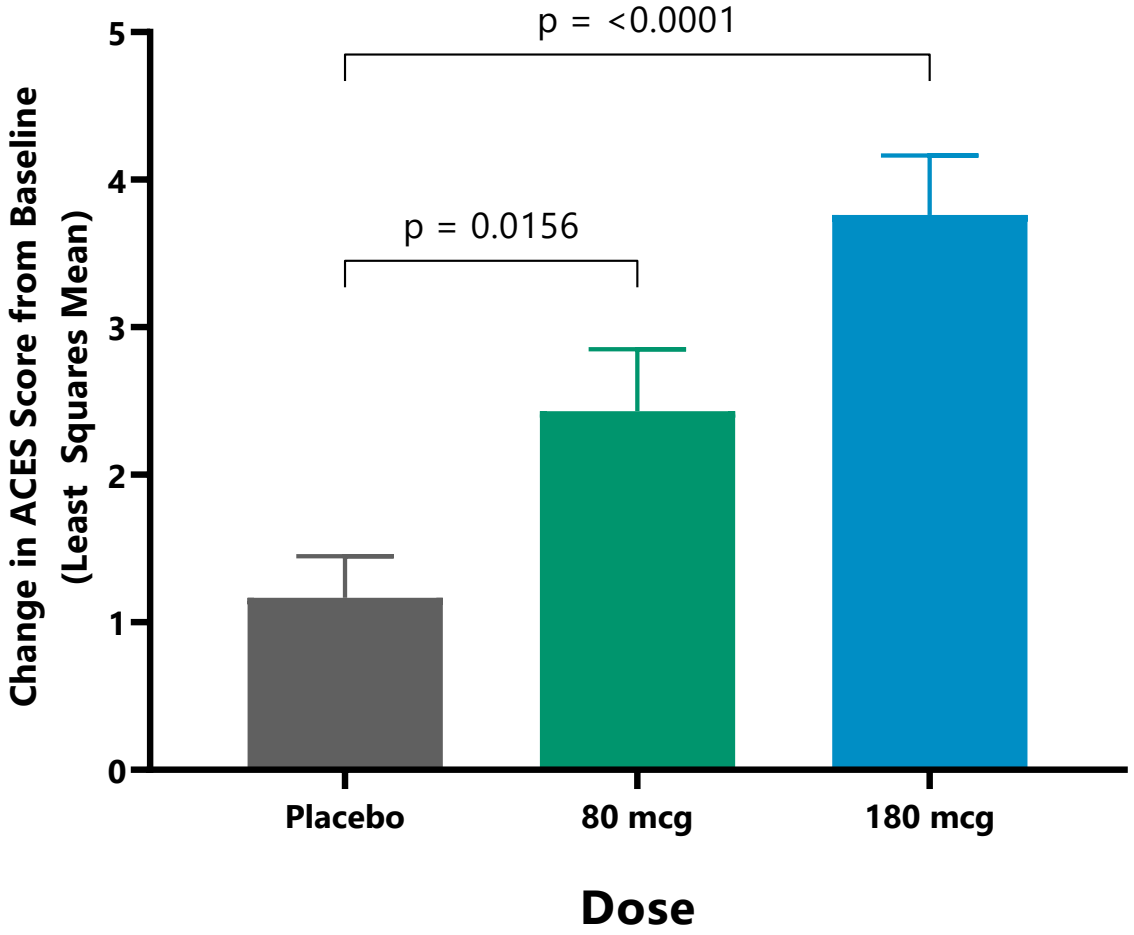
Time = 120 Min (Primary Endpoint)

Drug/Dose	#	% Responders (Reduction in PEC of ≥ 40%)	Mean Change in PEC Score	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

Secondary Evaluation: Change in ACES from Baseline

Consistent with primary endpoint



Drug/Dose	#	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

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.

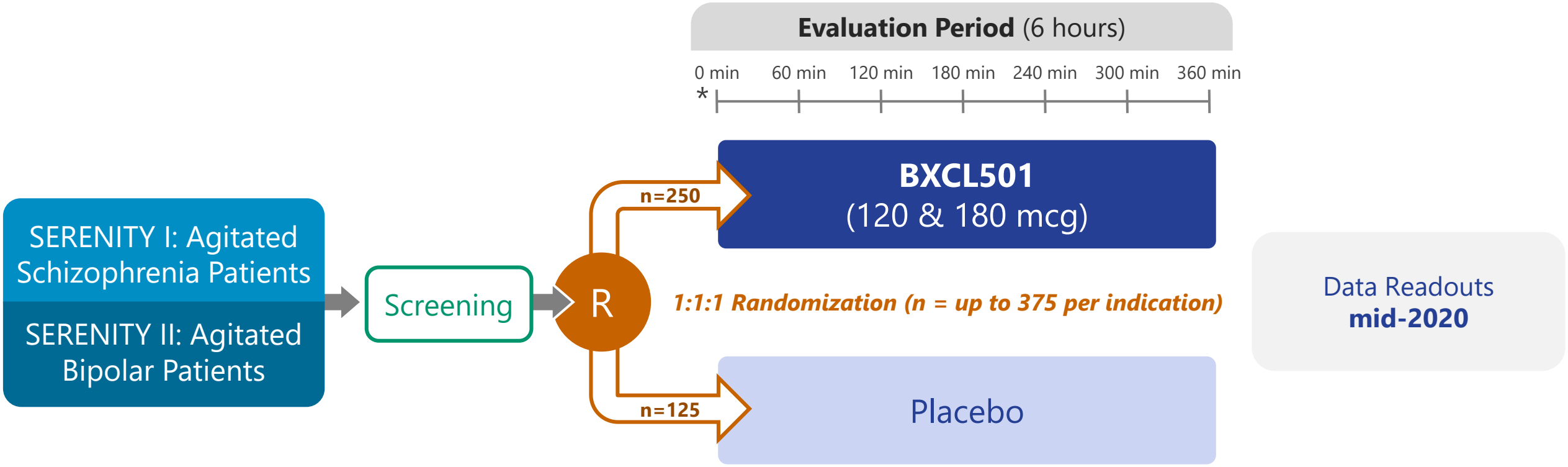
Phase 1b: Safety and Tolerability

- Well-tolerated with no serious or severe adverse events across the entire dose range tested.
- 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 completed the study.

Phase 3 Pivotal Trial: Adaptable Design

SERENITY:

Sublingual D**E**xmedetomidine in Agitation Associated with Schizoph**RENI**a and Bipolar Disorder S**T**ud**Y**

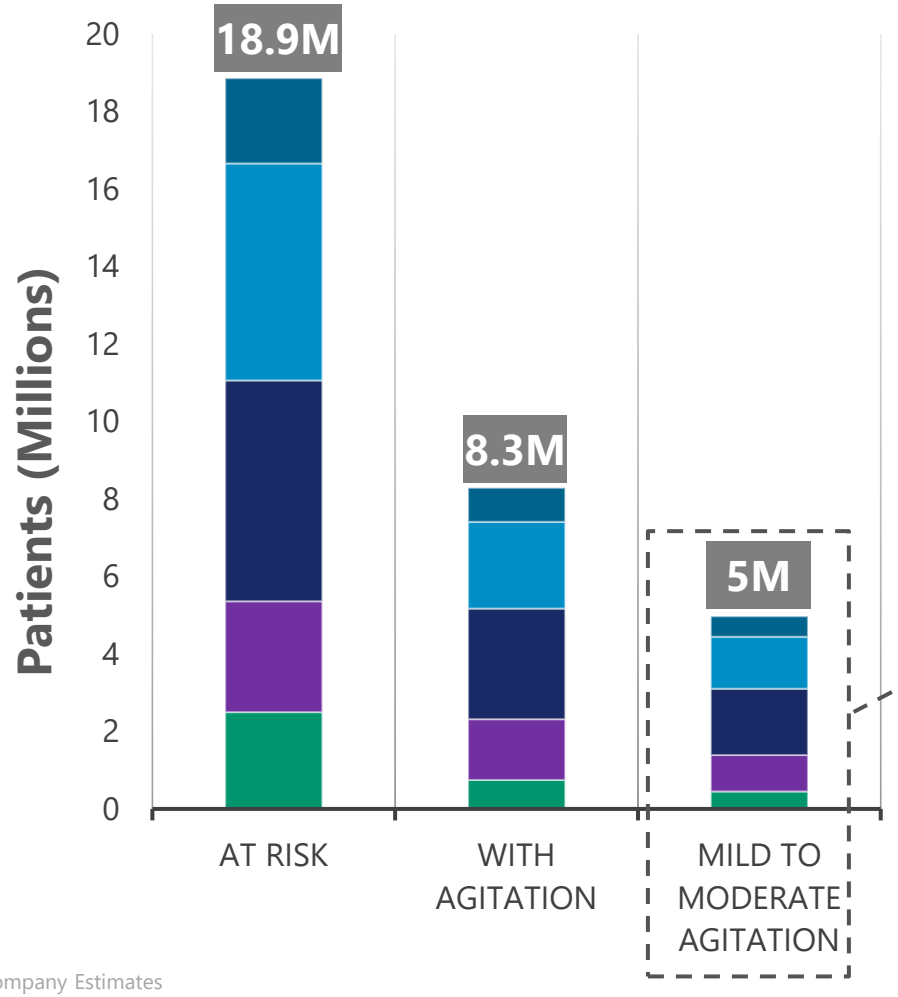


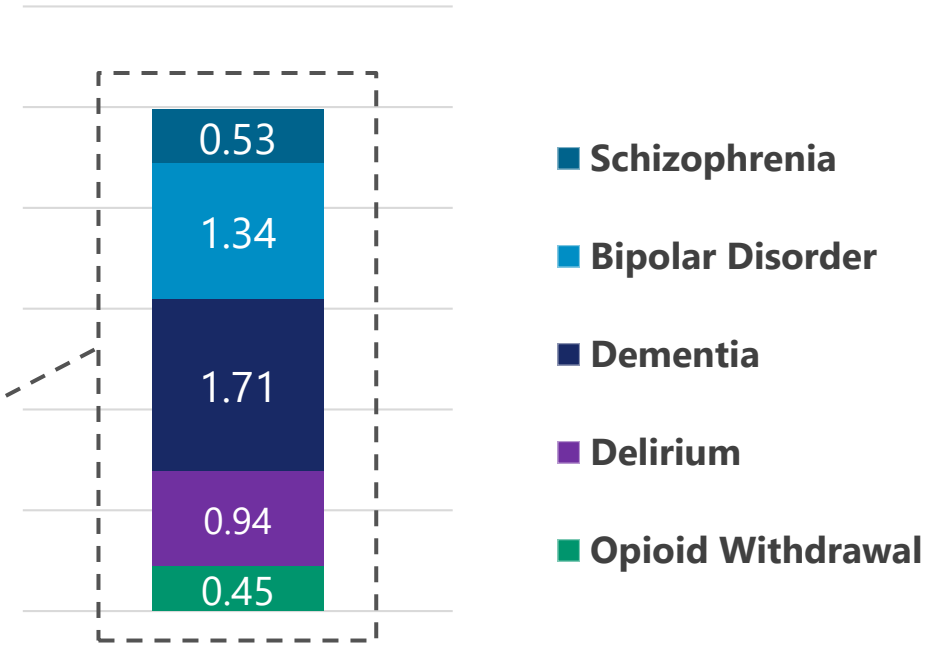
Primary Endpoint: Change from baseline in PEC score (PANSS-Excitatory Component)

* Patients Dosed

Potential To Capitalize on Large US Commercial Opportunity

- 5 Million Patients With Mild to Moderate Agitation
- Multiple Episodes Per Year





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

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Care Centers for Neuropsychiatric Indications

Broad Potential for Use Across Multiple Clinical Environments

Emergency Departments
General | Psychiatric

Urgent Care Centers

BXCL501

Hospitals
Medical + Neuro + Psych Wards
Psychiatric Hospitals

Mental Health Clinics
Crisis Care

Key Targeted Milestones for Value Creation

BXCL501
Anticipated Timeline

	3Q 2019	4Q 2019	mid-2020	2H 2020	2021
Schizophrenia / Bipolar Disorder	★ Phase 1b Data Announced (July 22, 2019)	Phase 3 Pivotal Trial Initiation Expected	★ Phase 3 Data Readout	★ First NDA Submission	★ Commercial Launch

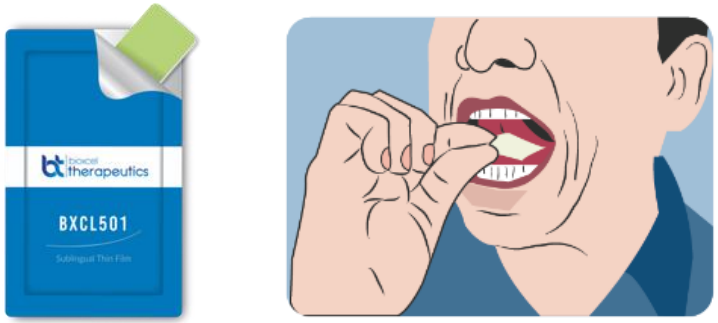
Pipeline in a Product and Agitation Franchise Opportunity

Pre-Agitation | **Mild to Moderate Agitation** | **Severe Agitation/ Aggression**

BXCL501 + Wearable Device Combo

Prophylaxis or Prevention of Agitation

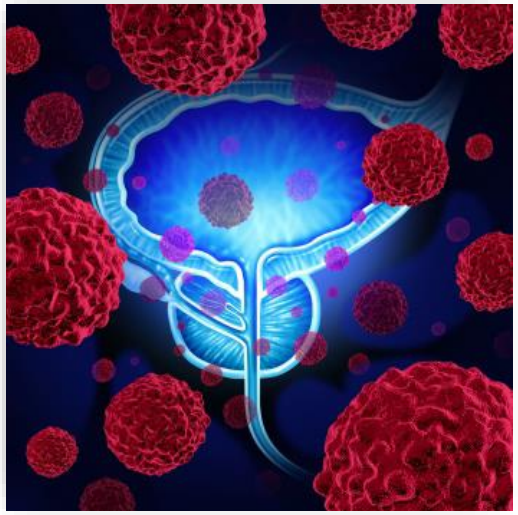
BXCL501 (Sublingual Thin Film)



- **Schizophrenia/Bipolar (NDA)**
- Dementia (sNDA)
- Opioid Withdrawal (sNDA)
- Delirium (sNDA)

KalmPen™

IM Formulation Development



Clinical Programs

BXCL701: Potential First-in-Class Oral IO Therapy Targeting Pancreatic Cancer and tNEPC

Oral I/O Therapy

Investigational Orally Administered Activator of Systemic Innate Immunity Pathway

Dual MoA Designed to Inhibit DPP 8/9 & FAP

Observed Proof of Mechanism in Clinical Studies

Orphan Drug Designations

FDA

- Pancreatic Cancer
- Melanoma
- Acute Myeloid Leukemia

nature chemical biology

Validation of AI Approach[1]

Clinical Partnership

Pfizer

NEKTAR

MERCK

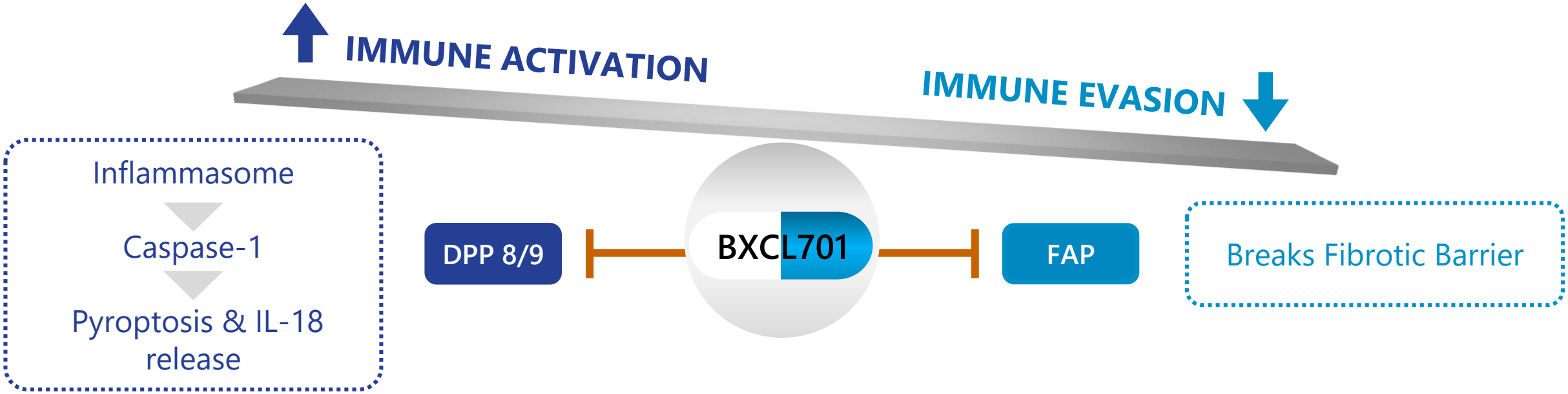
(1) *Nature Chemical Biology, volume 13, pages 46–53 (2017)*

Human Proof of Concept & Mechanism of Action

BXCL701 Human Proof of Concept

- ✓ Single Agent Activity in Melanoma
- ✓ ~10% Response Rate (CR/PR)

>700
Patient Data



tNEPC Clinical Development Plan: Combination with Keytruda

Safety Run-in
Safety/PD/Immune-phenotyping
(N=6)

→

Phase 2 Expansion
(N=30)

→

Global* Pivotal Study

*Expect to commence global development planning during Phase 2

Focus on EU and Japan

KEYTRUDA® (pembrolizumab)

✓ Multiple Patients Dosed

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)

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UCSF Health

Johann de Bono, M.D., Ph.D.
Head, Division of Clinical Studies
ICR The Institute of Cancer Research

tNEPC Phase 1b – Safety Run-In Summary

- Cohort 1: Enrollment and DLT Evaluation Period Complete
  - BXCL701 (0.4 mg/day) + Keytruda®
  - Well-tolerated with no SAEs or DLTs
  - Preliminary pharmacokinetics of BXCL701 are within expectations based on prior data
- Cohort 2: Enrollment Complete
  - BXCL701 (0.6 mg/day) + Keytruda®
  - Safety assessment is ongoing
  - Expected completion in Q4 2019

Pancreatic Cancer: Mechanistic and Triple Combination Trial

Proof of Mechanism Trial → 2 Weeks of **BXCL701** Treatment Before Surgery (Pre and Post Tissue Available (N=10-15))

Demonstration of Immune Cell Infiltration/Activation to Validate MoA

Efficacy Trial in Metastatic Patients after First-line Treatment → Triple Combination* Phase 2 Expansion (N=30)

BXCL701
NKTR-214
Avelumab

Activity
- NO → Stop
- YES → Global* Pivotal Study

Pfizer | MERCK | NEKTAR

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 Weiner, M.D.
Director
Georgetown | Lombardi COMPREHENSIVE CANCER CENTER

*BXCL701 phase expected to be initiated following Nektar and Pfizer's safety run-in trial of a double combination of NKTR-214 and avelumab and the outcome of that trial.

Key Targeted Milestones for Value Creation

BXCL701
Anticipated Timeline

Neuroendocrine Prostate Cancer (tNEPC)	Combination Trial Initiated (BXCL701+Keytruda)	Sites Opening & Recruitment Ongoing	★ tNEPC Data Readout		Registration Trial
Pancreatic Cancer (PDA)		Triple Combination Trial Initiation Mechanism Trial Initiated	Sites Opening & Recruitment Ongoing	★ Pancreatic Data Readouts	Registration Trial

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THANK YOU!