NASDAQ: BTAI

bioxcel therapeutics

Next Wave of Medicines Utilizing AI

May 2020

Forward-Looking Statements

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Overview

SERENITY Program Pivotal Readouts Expected in Mid-2020

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

- Phase 3 schizophrenia and bipolar trials (SERENITY I & II) initiated; readouts expected mid-2020
- Phase 1b/2 dementia trial (TRANQUILITY) initiated; readout expected mid-2020
- Phase 1b/2 opioid withdrawal trial (RELEASE); preparing to initiate

Immuno-oncology Program

BXCL701—Targeting Rare Cancers

- Phase 1b/2 double combo trial in Neuroendocrine Prostate Cancer (tNEPC) ongoing; Phase 2 efficacy portion initiated
- MD Anderson led Phase 2 basket trial in advanced solid tumors
- Phase 1b/2 triple combo trial in pancreatic cancer initiation expected 2020

Strengthened balance sheet in Feb. 2020 through follow-on offering raising $60 million in net proceeds

AI Platform May Reduce Development Timelines and Cost

4–5 Year Development Cycle

Multiple Candidates Screened By A I

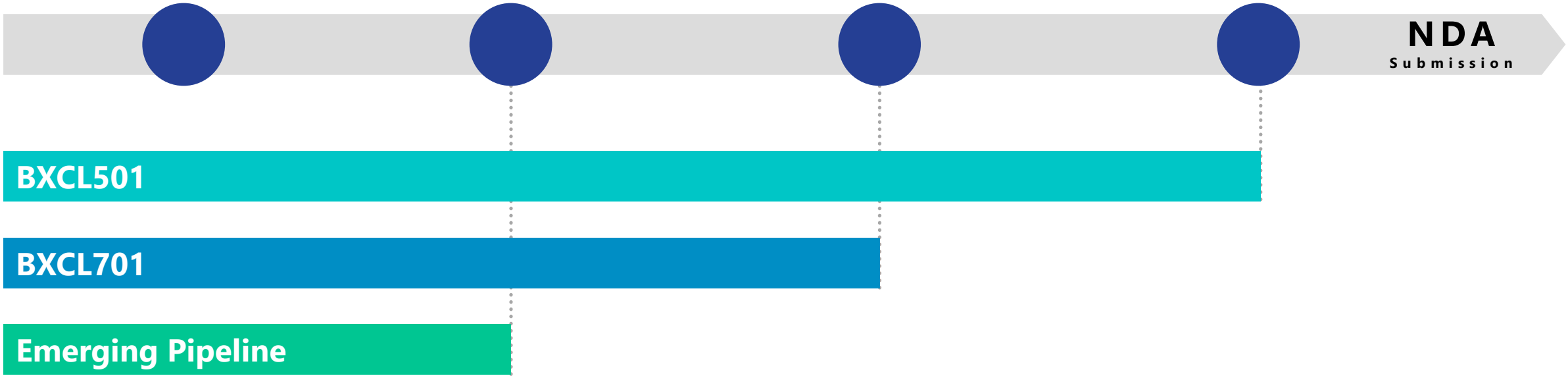
Selection of Best Candidates

Translational Team

Candidate Validation

Clinical Development Team & Regulatory Team

Human Proof of Concept & Registration Trials



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Pipeline

Neuropsychiatry

BXCL501	
Acute agitation in schizophrenia/bipolar	**SERENITY I & II Trials (Phase 3)**
Acute agitation in dementia	**TRANQUILITY Trial (Phase 1b/2)**
Opioid withdrawal	**RELEASE Trial (IND Clearance)**
Delirium	**Clinical Planning**
KalmPen™ (Single-use IM)	
Severe agitation	**Formulation Development**
Wearable Device (+BXCL501)*	
Pre & post-agitation in dementia	**Clinical Feasibility Study**
BXCL501 + combination	
Chronic agitation in dementia	**Formulation Development**

Immuno-oncology

BXCL701	
Neuroendocrine Prostate Cancer (tNEPC) Double Combination	**Phase 2**
Advanced Solid Tumor Types (MD Anderson Led)	**Phase 2**
Pancreatic Cancer Triple Combo	**Phase 1b/2**

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

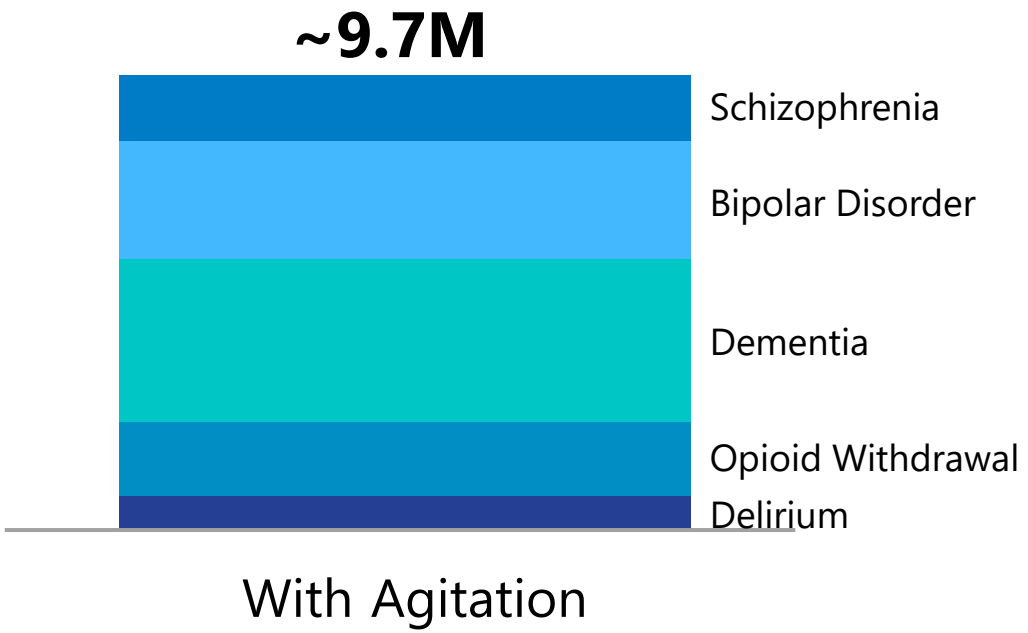
BXCL501:

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

Agitation: A Common Phenomenon Associated with Psychiatric Conditions

High Unmet Medial Need in the U.S.

- 9.7 million suffer each year [1]
  - Schizophrenia/bipolar: 3.1M
  - Dementia: 4M
  - Opioid withdrawal: 1.6M
  - Delirium: 1M [2]
- Patients experience multiple episodes per year
- $40 billion per year health care burden
- Agitation episodes can put both the patient and caregiver at risk



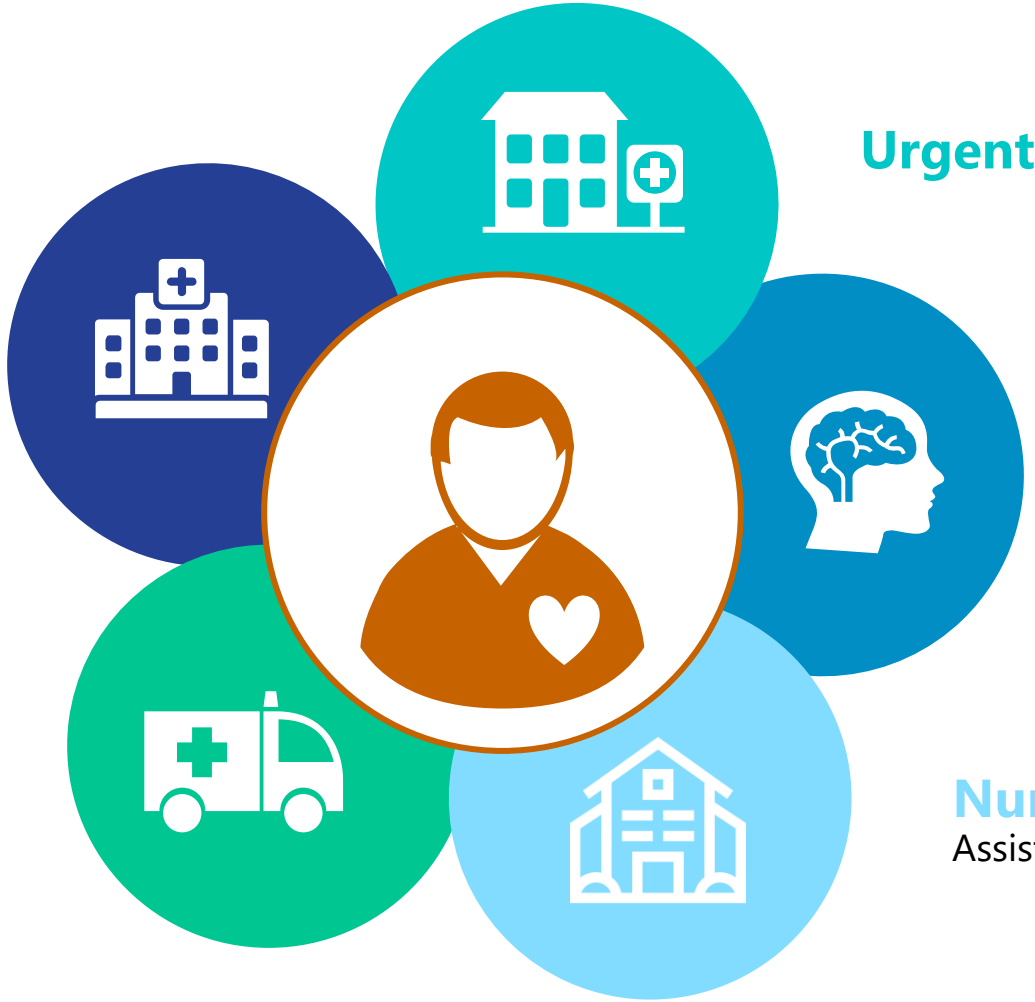
1. Internal company estimates based on analysis of primary market research, prescription database, and published data.
2. Agitated Delirium in ICU, does not include hyperactive delirium in medical and surgical wards

Broad Market Potential Across Centers

Where Neuropsychiatric Patients are Treated

Hospitals
Medical + Neuro + Psych Wards
Psychiatric Hospitals

Emergency Departments
General, Psychiatric



Urgent Care Centers

Mental Clinics
Neurology/Mental Health
Crisis Care

Nursing Homes
Assisted Living

Current Treatment Options Fail to Address the Underlying Condition

Current Therapies Are Suboptimal

- Verbal de-escalation is used as first line treatment
- Injectables are invasive with severe side effects
- Antipsychotic drugs have black-box warning for elderly
- Restraining can damage the caregiver/patient relationship
  - Requires 1:1 observation
- Over-sedation is a major issue – patients cannot be properly evaluated

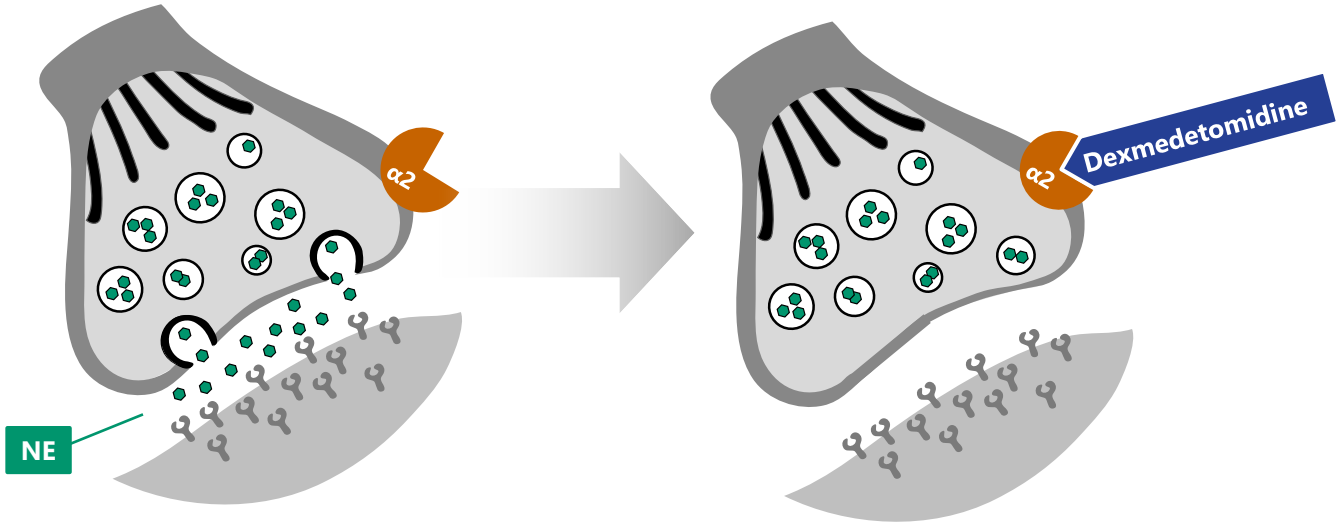
BXCL501's product profile offers significant advantages over the standard of care

Fast Track Designation

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

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 formulation
- ✔ Non-traumatic
- ✔ Rapid onset of action, without excessive sedation (observed in clinical studies)
- ✔ Non-invasive
- ✔ Self-administered by patients

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Proprietary, Easy-to-Administer Formulation

Proprietary, Immediate Delivery, Sublingual Thin Film Product

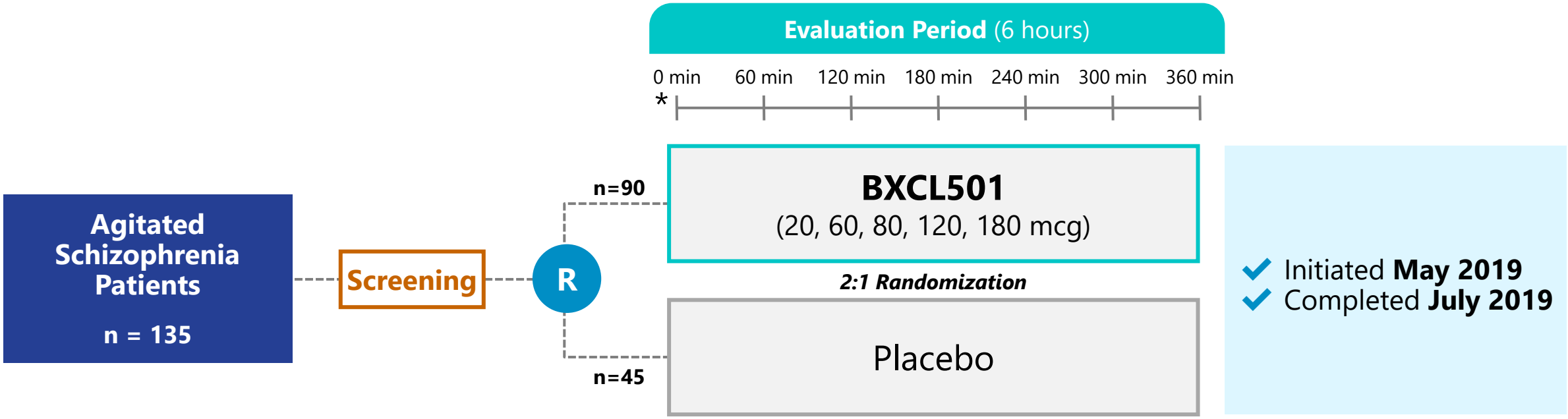
- Muco-adhesion properties designed for optimizing compliance
- Adaptable technology enables broad dose range
- Flexible for potential combination of multiple drugs on a single film
- Expected patent term until 2039 -2041

Transitioned to Registrational Drug Product Process

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

Successful Phase 1b Clinical Trial in Agitated Schizophrenia Patients

Assessing Agitation Episodes in Schizophrenia

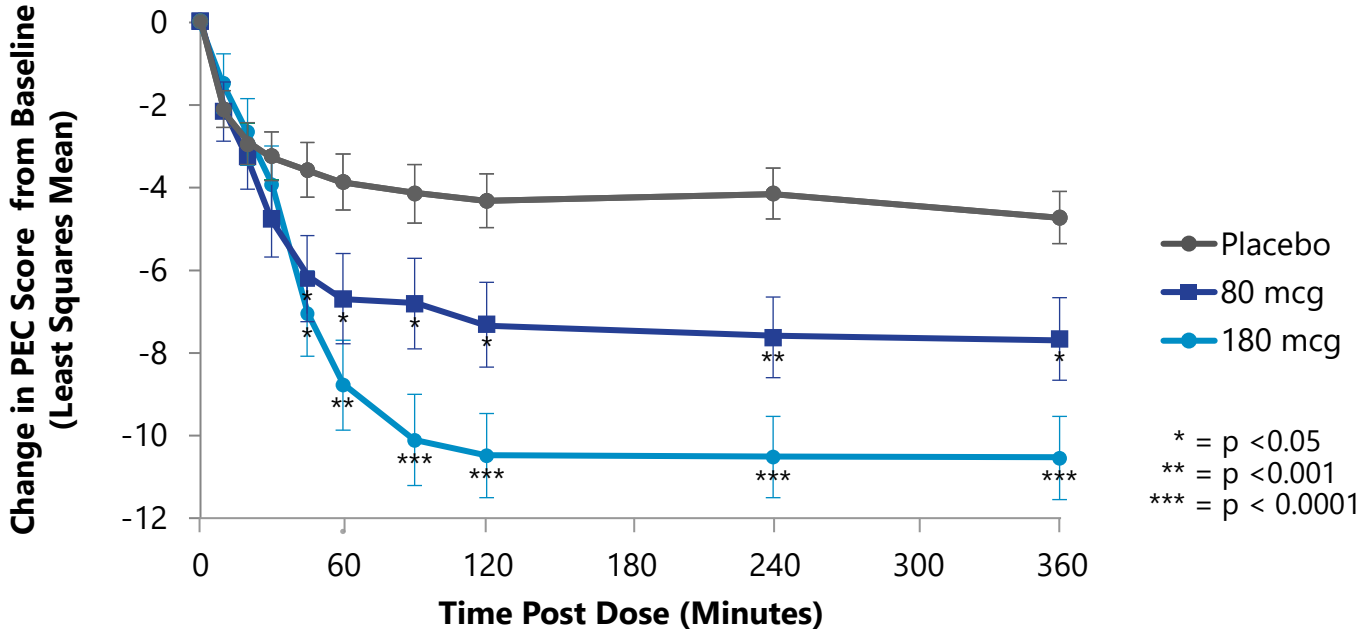


Primary Endpoint: Change from Baseline in PEC Score (PANSS-Excitatory Component) at 2 Hours

* Patients Dosed

Primary Endpoint: Statistically Significant Change in PEC Score

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

Safety Results in Phase 1b Study

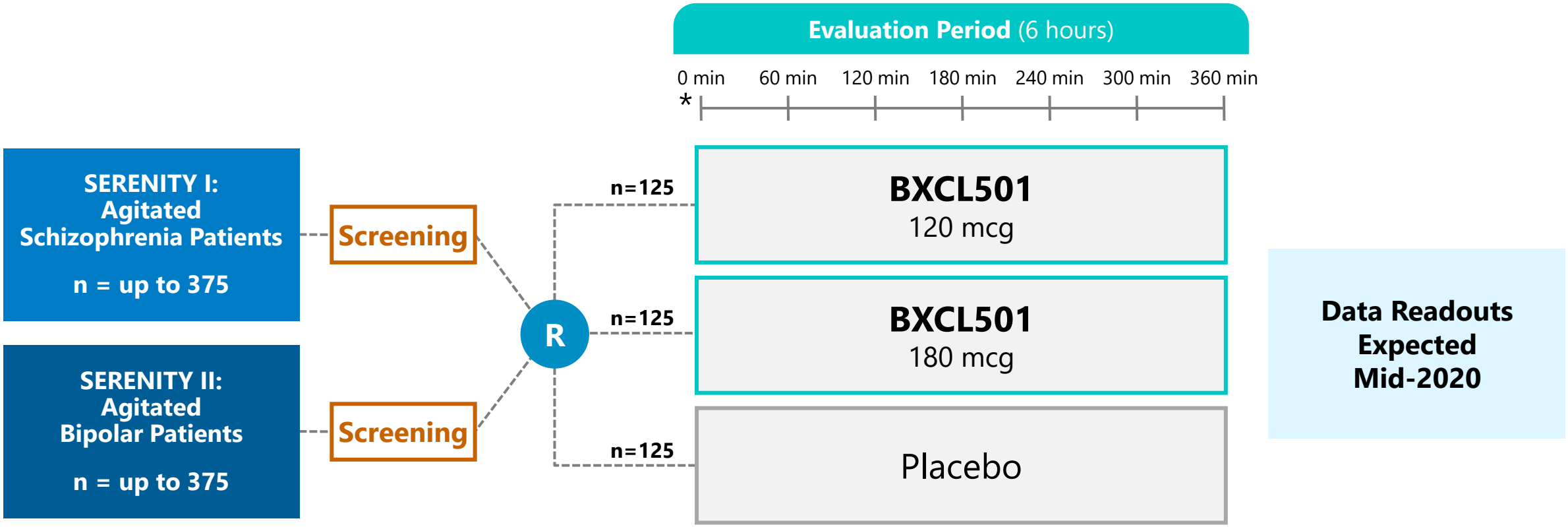
Well-tolerated with no serious or severe adverse events

Most common adverse events: mild somnolence and dry mouth

Maximum tolerated dose was not reached

All subjects (100%) were able to self-administer the film

SERENITY Phase 3 Pivotal Trials Initiated: Adaptive Design

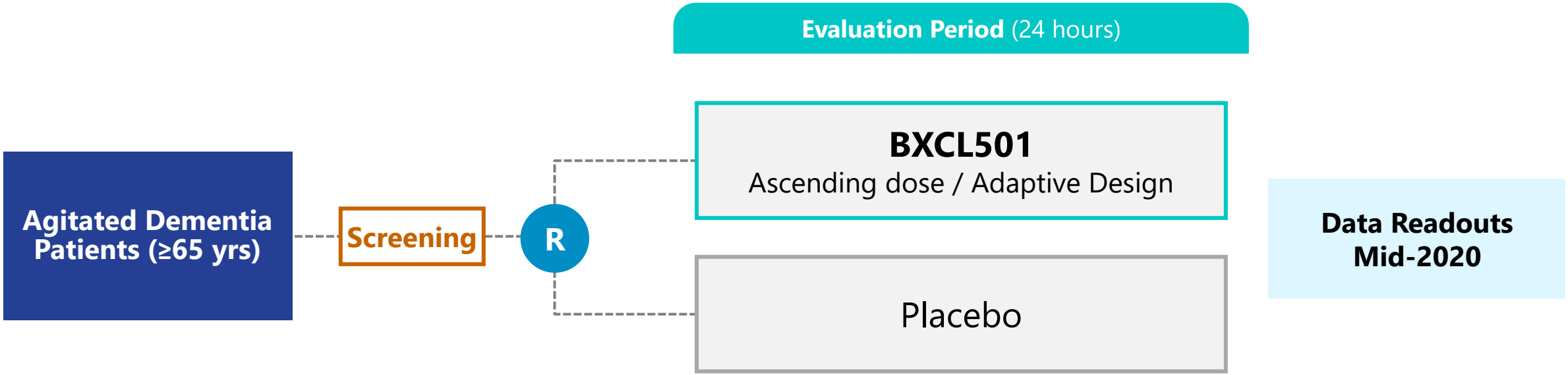


Primary Endpoint: Change from Baseline in PEC Score (PANSS-Excitatory Component) at 2 Hours

* Patients Dosed

TRANQUILITY Phase 1b/2 Trial Initiated – Dementia

Assessing Agitation Episodes in Dementia



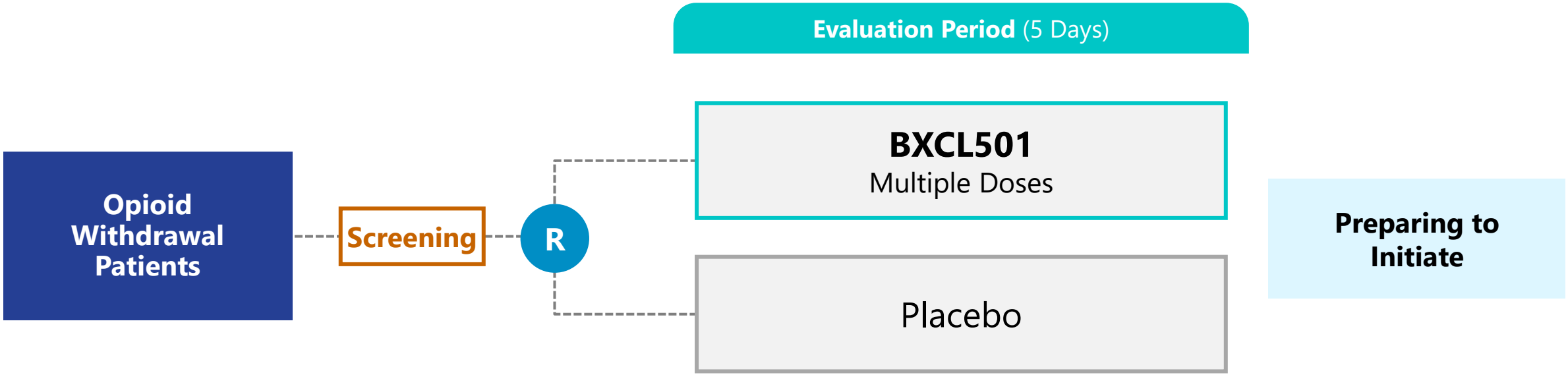
Key Inclusion Criteria

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

Efficacy Endpoints

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

RELEASE Phase 1b/2 Trial – Opioid Withdrawal



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

Efficacy Endpoints

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

Neuroscience Program Strategy

Preagitation

Prophylaxis or Prevention of Agitation

Mild to Moderate Agitation

Schizophrenia/Bipolar (NDA)

Dementia (sNDA)

Opioid Withdrawal (sNDA)

Delirium (sNDA)

Severe Agitation/Aggression

IM Formulation Development

BXCL501 Product Development Milestones

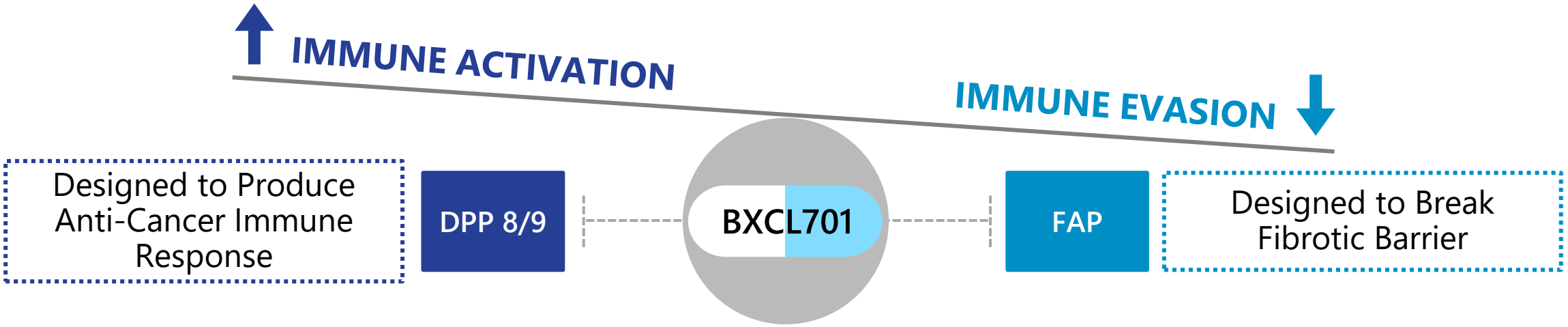
	4Q2019	1Q2020	Mid-2020	2021
SERENITY I & II Trials (Schizophrenia/Bipolar)	Phase 3 Pivotal Trial Initiation		Expected Phase 3 Data Readout	Expected NDA Submission
TRANQUILITY Trial (Dementia)	Phase 1b/2 Trial Initiation		Expected Phase 1b/2 Data Readout	
RELEASE Trial (Opioid Withdrawal)		Phase 1b/2 Trial IND Clearance		

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



- Current approved immunotherapies struggle to address tumors that appear “cold” or uninflamed
- BXCL701 is designed to stimulate the innate immune system, facilitating a strong adaptive anti cancer immune response – turning “cold” tumors “hot”

BXCL701 Clinical Development Strategy

Multiple Opportunities to Evaluate Patient Outcomes

1. **Treatment Emergent Prostate Cancer (tNEPC):** Phase 1b/2 trial of BXCL701 and KEYTRUDA

2. **Pancreatic Cancer:** Triple combination (BXCL701, bempegaldesleukin and BAVENCIO®) Phase 1b/2 trial

3. **Solid Tumors Responsive to CPIs*:** Open-label Phase 2 basket trial led by MD Anderson

Orphan Drug Designations

FDA

- Pancreatic Cancer
- Melanoma
- Acute Myeloid Leukemia

Clinical Partnership

Pfizer

NEKTAR

MERCK

* CPI: Check Point Inhibitors

Phase 1b/2: In Combination with KEYTRUDA to Treat tNEPC

1

Completed Safety Run-in*
Safety/PD/Immune-phenotyping

KEYTRUDA® (pembrolizumab)

- Identified recommended Phase 2 dose of BXCL701 when used in combination with KEYTRUDA®
  - 0.3 mg of BXCL701 twice daily (BID)
- This dose has shown on-target side effects consistent with cytokine activation

Initiated Phase 2 Efficacy Portion
(N=30)

- **Simon 2-stage:**
  15+15
- **Primary Endpoint:**
  Composite response rate: Target > 15%
- **Secondary Endpoint:**
  DoR, PFS, OS
- **Exploratory Endpoint:**
  Effect on immune cells (MDSC, T-cells, neutrophils)
- Initial interim data readout expected in Q4 2020

* Initial safety data presented at The 26th Annual Prostate Cancer Foundation Scientific Retreat and at ASCO GU

Pancreatic Cancer: Triple Combination Trial

2

Triple Combination*
Phase 2 Expansion (n=30)

BXCL701
NKTR-214
Avelumab

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

*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.

Expanding Study of BXCL701: Open-label Basket Trial with Keytruda

3

CPI Responsive Solid Tumor Study

KEYTRUDA (pembrolizumab)

- Cohort A: evaluating patients who are naïve to checkpoint therapy
- Cohort B: evaluating patients who have failed or are refractory to checkpoint therapy
- **Outcome measures:** ORR, progression-free-survival, overall survival, duration of response, and the safety of combined treatment

THE UNIVERSITY OF TEXAS MD Anderson Cancer Center

Making Cancer History®

BXCL701 Program Milestones

	1H2019	2H2019	1H2020	2H2020
Neuroendocrine Prostate Cancer (tNEPC)	Site Activation & Recruitment Ongoing		Phase 2 Efficacy Portion Initiated	Expected Initial Interim Data Readout
Pancreatic Cancer (PDA)	Mechanism Trial Initiated		Double Combination Trial Ongoing	Expected Triple Combination Trial Initiation Expected Initial Mechanistic Data Readouts
Solid Tumors Responsive to CPIs*			Expected Phase 2 Basket Trial Initiation	Expected Initial Data Readout

*MD Anderson Led IST

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Thank You!

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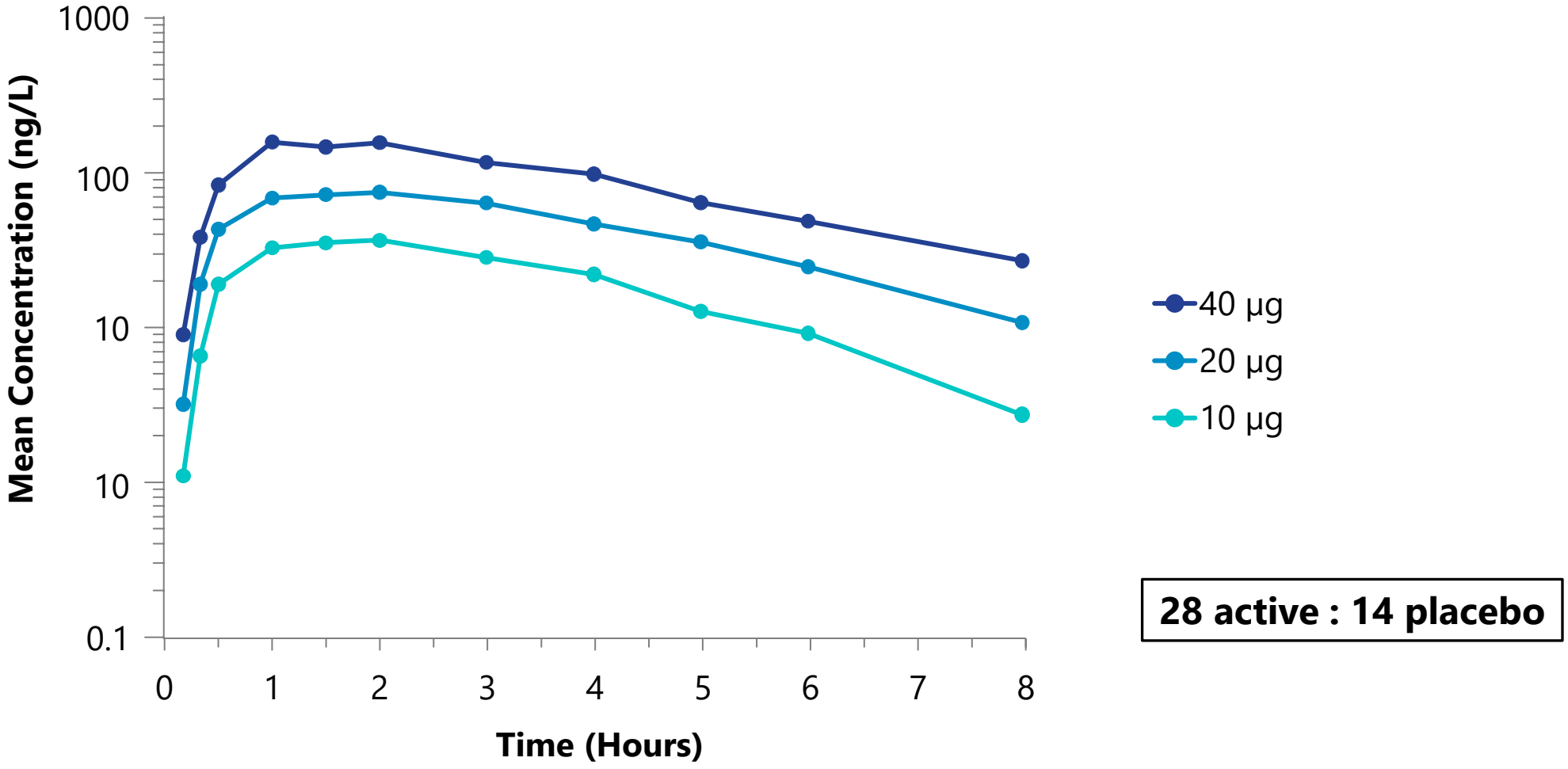
Appendix

Predictable and Dose Proportional PK Observed in Phase I Study

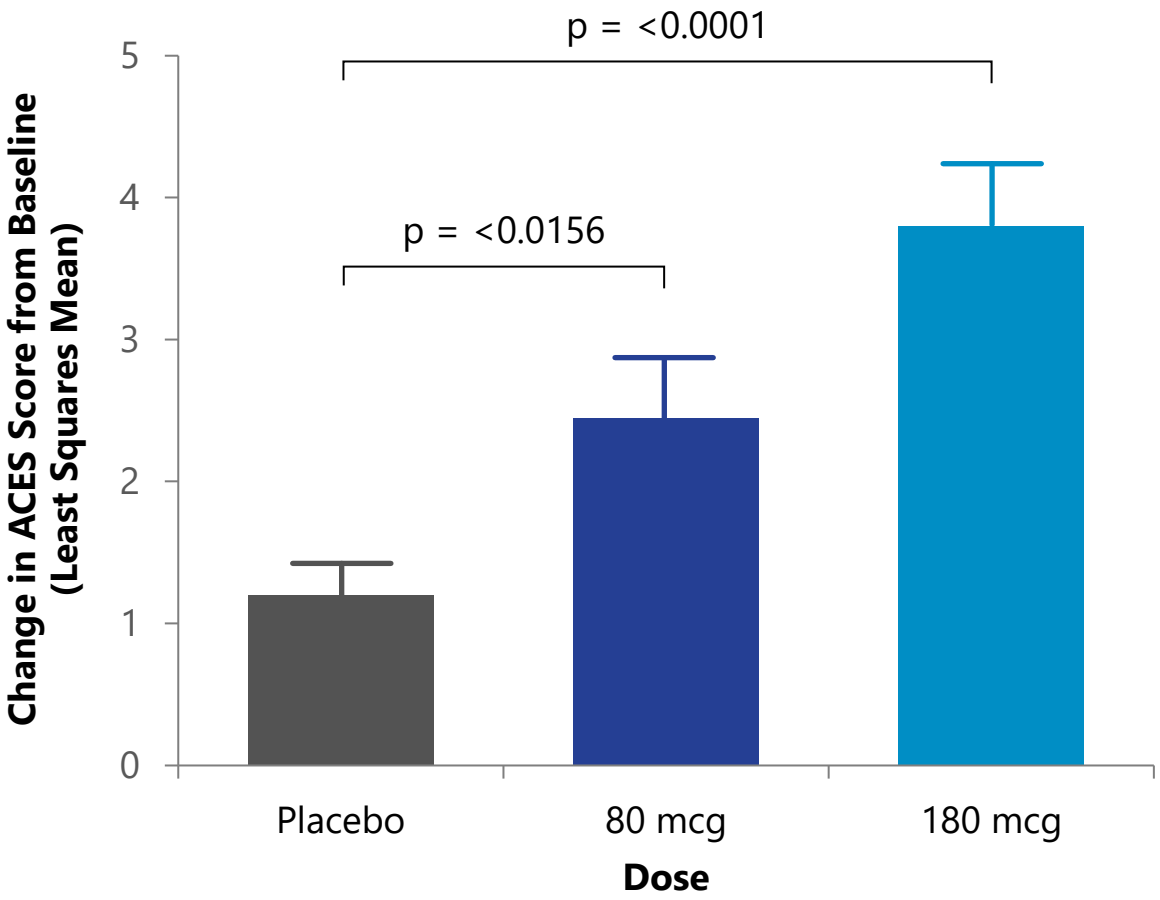
Phase I Clinical Studies in 42 Healthy Volunteers



28 active : 14 placebo

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.