Phase 3 SERENITY I & II Trials – Topline Results

Acute Treatment of Agitation in Patients with Schizophrenia and Bipolar Disorder

July 20, 2020

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Agenda

Topic	Presenter
Overview & Summary	Vimal Mehta, Ph.D., CEO & Founder
SERENITY Trial Design & Results	Robert Risinger, M.D., VP, Clinical Development
Commercial Planning	William Kane, EVP & CCO
Summary & What's Ahead	Vimal Mehta, Ph.D., CEO & Founder
Q&A	BioXcel Therapeutics Team

Agitation: Debilitating for Patients and Threatening for Healthcare Providers

A common and difficult to manage symptom

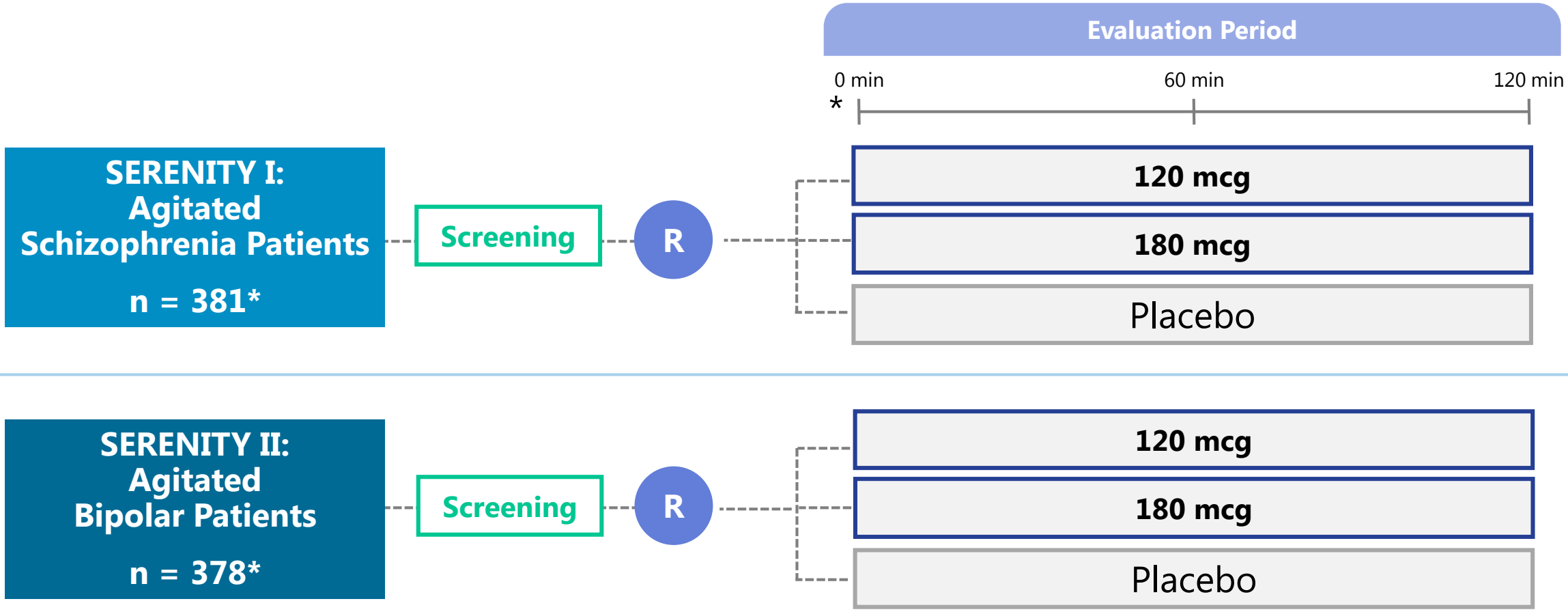
- Agitation is a common occurrence in most neuropsychiatric disorders
- Characterized by recurring episodes requiring frequent treatments
- Affects over 65 million people worldwide resulting from schizophrenia and bipolar disorders
  - ~ 8M patients in the U.S. and 40% experience agitation
- Current treatment options are suboptimal
  - Physically restraining patients
  - Over-sedating therapies such as antipsychotic and benzodiazepines
  - Antipsychotic drugs have black box warning for elderly
- BXCL501 offers a novel mechanism and a highly differentiated approach

Robust Treatment Effect Observed With BXCL501

- ✓ Highly statistically significant improvements in PEC score observed vs. placebo ($p<0.0001$) at two hours in the SERENITY trials for both doses tested
- ✓ Statistically significant improvements in PEC score observed as early as 20 minutes after treatment
- ✓ All exploratory endpoints demonstrated statistically significant and clinically meaningful reductions in agitation measures that were durable
- ✓ BXCL501 was well tolerated with no serious adverse events
- ✓ NDA submission to U.S. FDA planned for Q1 2021

SERENITY I & II Trial Design

SERENITY I & II: Two Pivotal Phase 3 Trials Evaluating BXCL501



Primary Endpoint: Change from Baseline in PEC Score (PANSS-Excitatory Component) at 2 Hours
Secondary Endpoint: Earliest Time Where an Effect on Agitation is Apparent

* Patients Dosed

Exclusion/Inclusion Criteria Similar Across Both Studies

Inclusion Criteria included:

- SERENITY I: Schizophrenia
  - Diagnosis of schizophrenia, schizoaffective, or schizophreniform disorder
- SERENITY II: Bipolar Disorder
  - Diagnosis of bipolar I or II disorder
- Male and female 18-75 years of age, inclusive
- Total score of ≥ 14 on the 5 items comprising the PANSS Excited Component (PEC) at screening and baseline
- Score of ≥ 4 on at least 1 of the 5 PEC items at baseline

Exclusion Criteria included:

- Patients with agitation caused by acute intoxication
- Use of benzodiazepines, other hypnotics or antipsychotics 4 hours before study treatment

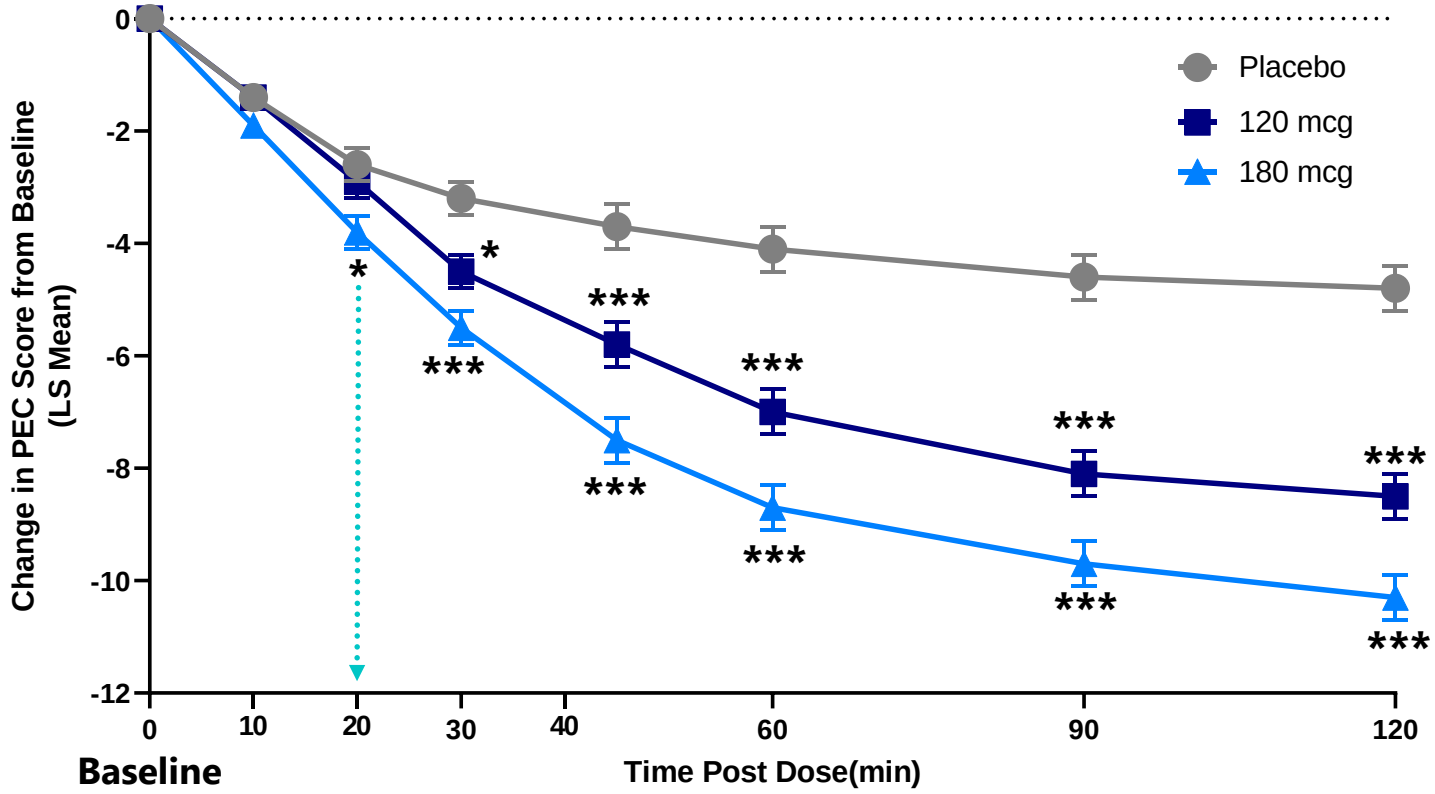
SERENITY I (Schizophrenia) Efficacy Results

SERENITY I: Demographics and Baseline Characteristics

		180 mcg BXCL501 (N=126)	120 mcg BXCL501 (N=129)	Placebo (N=126)	Overall (N=381)
Mean age (SD)		46.0 (11.91)	45.7 (11.32)	45.1 (11.13)	45.6 (11.43)
Female N (%)		44 (34.9)	52 (40.3)	44 (34.9)	140 (36.7)
Race (% white/ % non-white)		16.7/83.3	25.6/74.4	16.7/83.3	19.7/80.3
BMI		32.53 (7.9)	31.24 (7.6)	32.56 (7.4)	32.10 (7.6)
Diagnosis	Schizophrenia	80.2%	87.6%	85.7%	84.5%
	Schizoaffective	19.8%	12.4%	14.3%	15.5%
Baseline PEC means		17.6	17.5	17.6	Range (14 – 27)

98% of randomized patients completed trial

SERENITY I: Rapid Onset of Action Observed



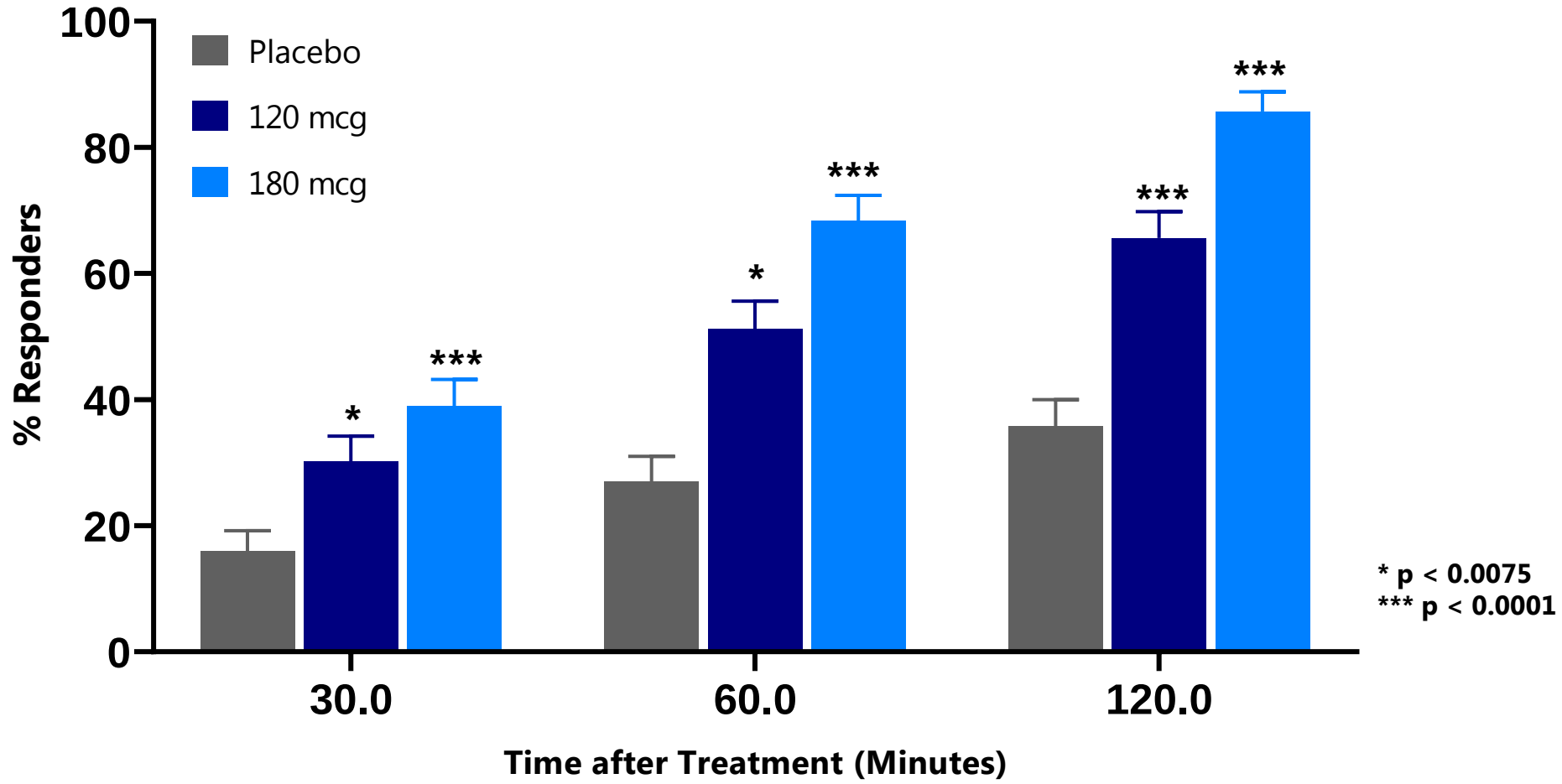
* p < 0.025
*** p < 0.0001

Primary Endpoint at 120 Min	Endpoint (120 min)	Placebo	120 mcg	180 mcg
	PEC Total score Change from Baseline	-4.8	-8.5 ***	-10.3 ***
	Response °	34%	67% ***	87% ***

ITT analysis, Least Square Means +/-SEM
° Proportion achieving ≥ 40% PEC reduction

SERENITY I: Clinically Meaningful Improvement Confirmed by CGI-I

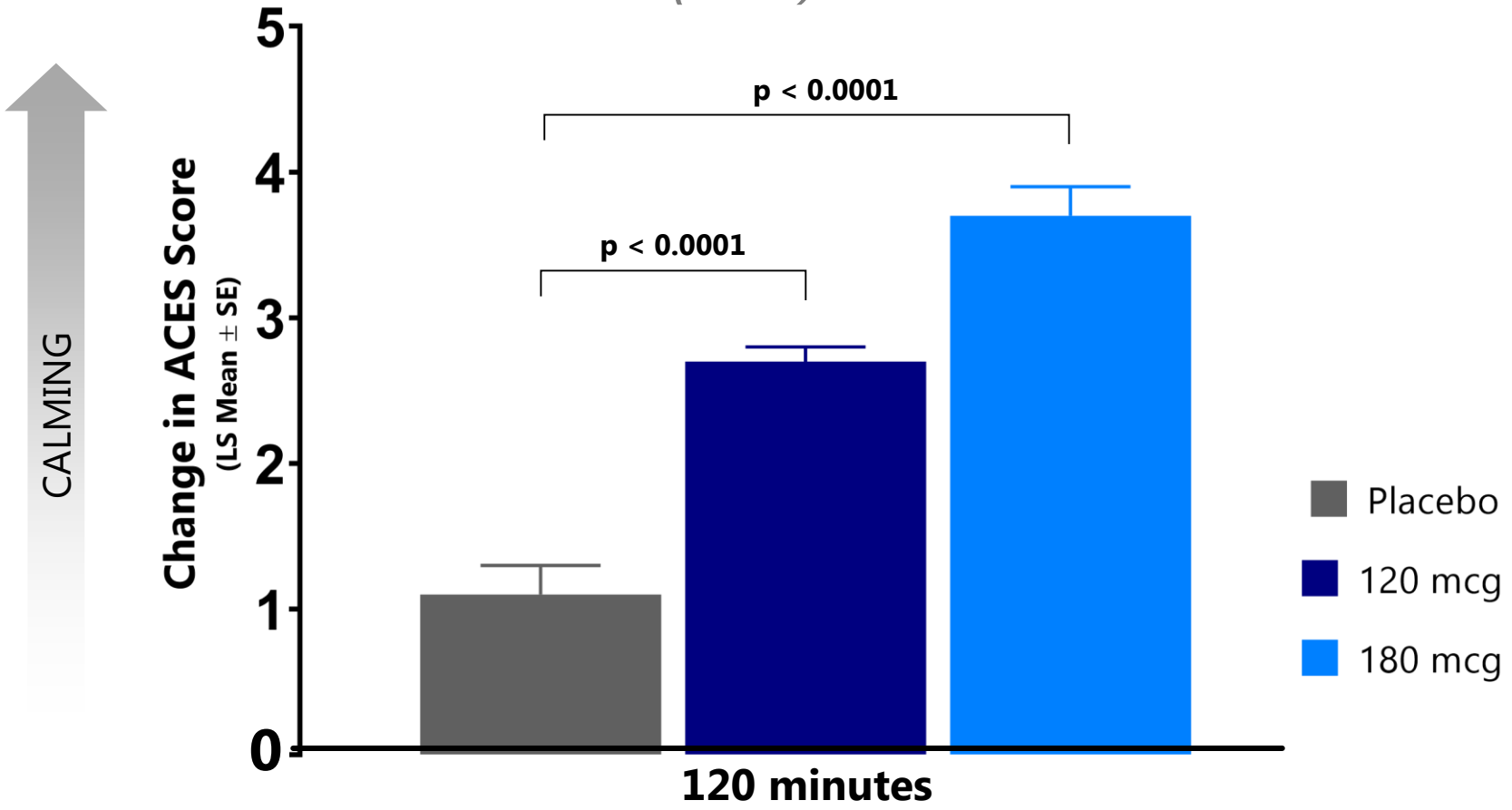
Measured by Clinical Global Impression – Improvement Scale (CGI-I)



The Clinical Global Impression scale – Improvement (CGI-I) is a 7-point scale. Ratings of (1) Very Much, or (2) Much Improved were considered Responders.
ITT analysis

SERENITY I: Independent Confirmation of Calming by ACES

Agitation and Calmness Evaluation Scale (ACES)



Significant calming observed at both doses

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.

ITT analysis

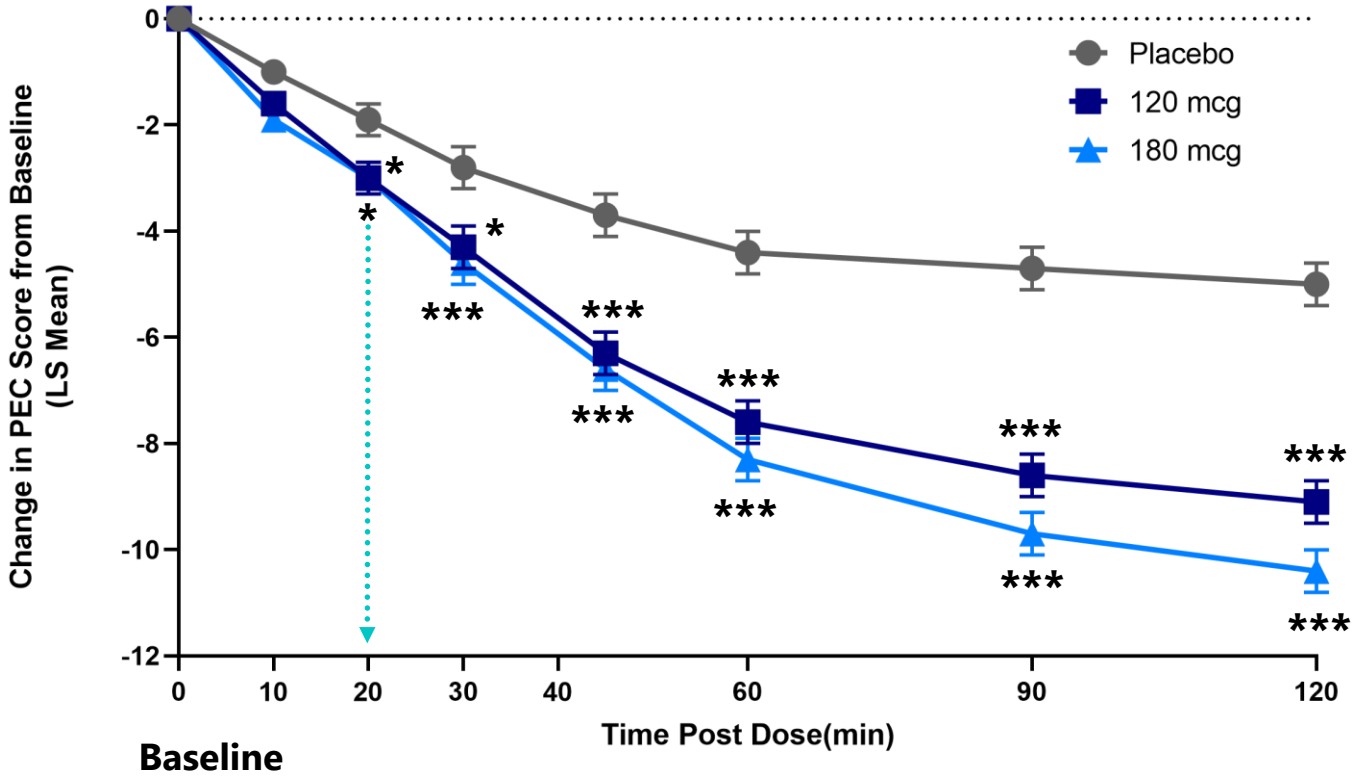
SERENITY II (Bipolar Disorder) Efficacy Results

SERENITY II: Demographics and Baseline Characteristics

		180 mcg BXCL501 (N=126)	120 mcg BXCL501 (N=126)	Placebo (N=126)	Overall (N=378)
Mean age (SD)		45.9 (11.30)	46.1 (11.53)	44.8 (12.05)	45.6 (11.61)
Female N (%)		67 (53.2)	67 (53.2)	73 (57.9)	207 (54.8)
Race (% white/% non-white)		38.9 /61.1	44.4/55.6	39.7/60.3	41.0/59
BMI		33.27 (8.7)	31.62 (8.0)	32.50 (7.4)	32.46 (8.0)
Diagnosis	Depressed	22%	16%	21%	20%
	Hypomania	4%	11%	8%	8%
	Mania	47%	46%	50%	48%
	Mixed Episodes	24%	21%	18%	21%
	Unspecified	3%	6%	4%	4%
Baseline PEC		18	18	17.9	Range (14 – 30)

95% of randomized patients completed trial

SERENITY II: Rapid Onset of Action Observed

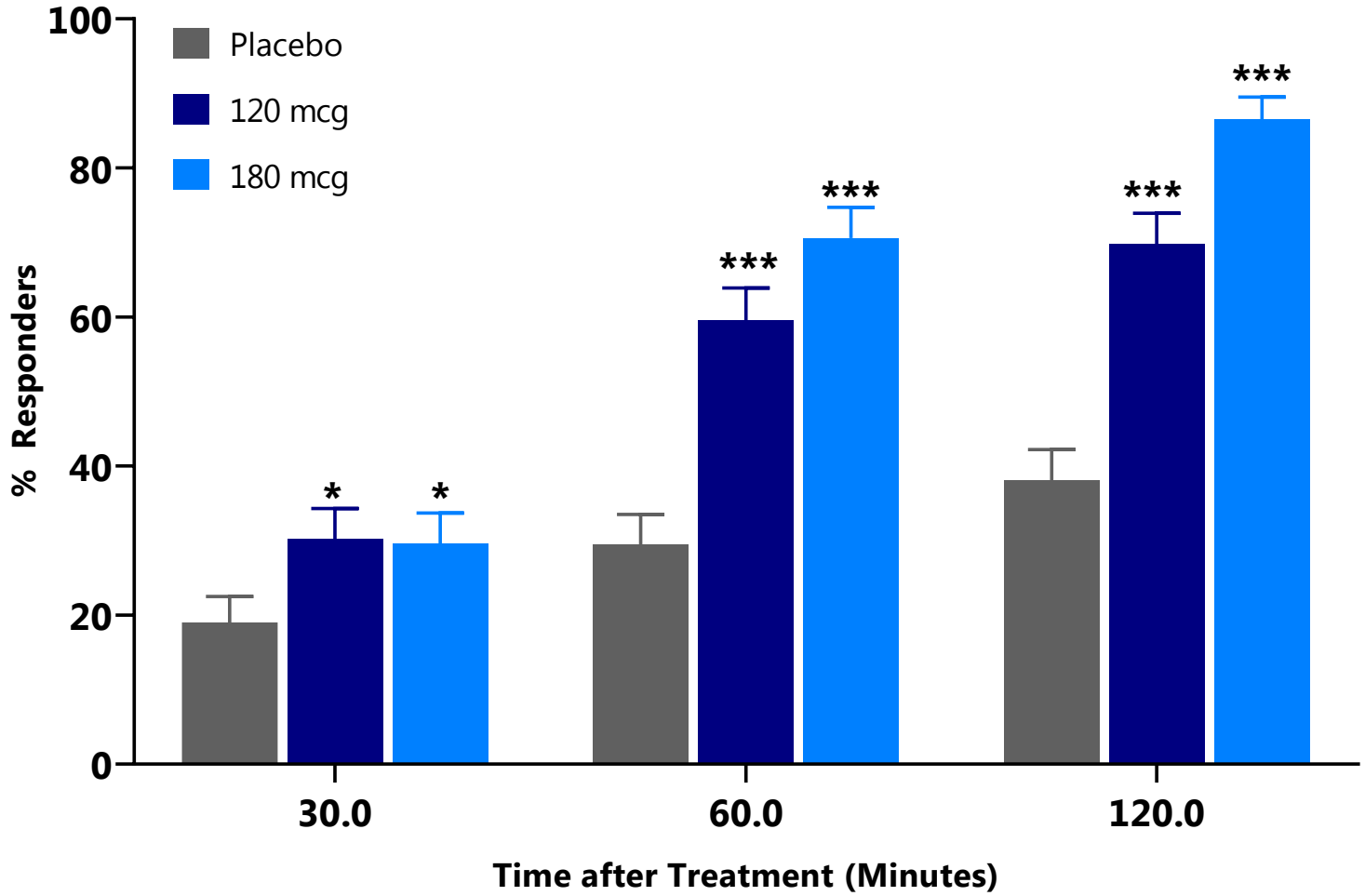


Time = 120 Min (Primary Endpoint)	Endpoint (120 min)	Placebo	120 mcg	180 mcg
	Primary: PEC total score change from Baseline	-5.0	-9.1 ***	-10.4 ***
	Response °	37%	69% ***	85% ***

ITT analysis, Least Square Means +/-SEM
° Proportion achieving ≥ 40% PEC reduction

SERENITY II: Clinically Meaningful Improvement Confirmed by CGI-I

Measured by Clinical Global Impression – Improvement Scale (CGI-I)

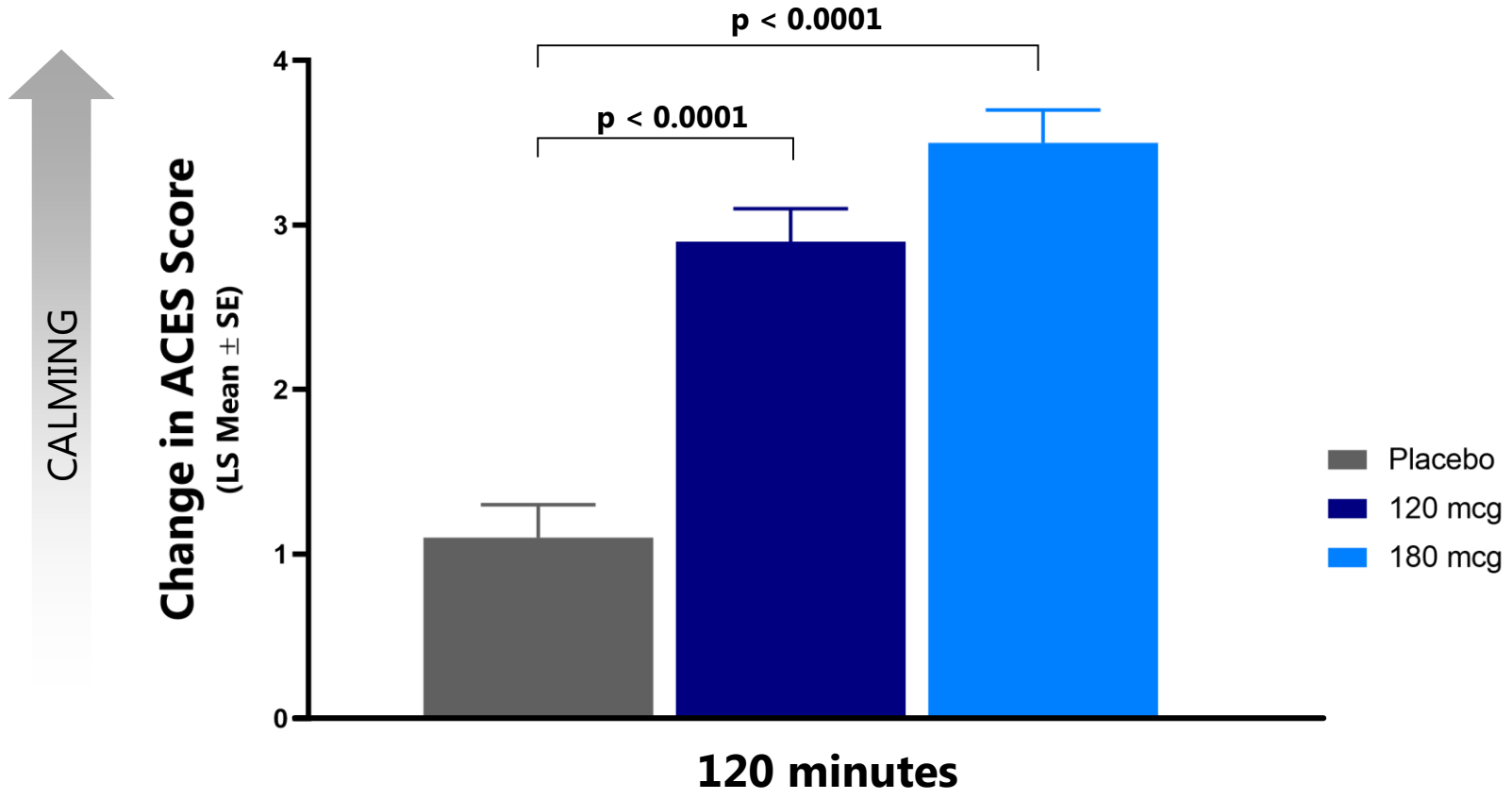


* p = 0.0567
** p = 0.0066
*** p < 0.0001

The Clinical Global Impression scale – Improvement (CGI-I) is a 7-point scale. Ratings of (1) Very Much, or (2) Much Improved were considered Responders.
ITT analysis

SERENITY II: Independent Confirmation of Calming by ACES

Agitation and Calmness Evaluation Scale (ACES)



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.

ITT analysis

BXCL501 Well Tolerated with No Serious Adverse Events

Tolerability results were comparable in both SERENITY trials

		180 mcg BXCL501 (N=252)	120 mcg BXCL501 (N=255)	Placebo (N=252)
Somnolence	Mild	40 (15.9)	43 (16.9)	15 (6.0)
	Moderate	16 (6.3)	11 (4.3)	1 (0.4)
Dizziness	Mild	13 (5.2)	7 (2.7)	2 (0.8)
	Moderate	2 (0.8)	3 (1.2)	0
Hypotension	Mild	10 (4.0)	10 (3.9)	0
	Moderate	3 (1.2)	4 (1.6)	0
Orthostatic hypotension	Mild	9 (3.6)	7 (2.7)	1 (0.4)
	Moderate	4 (1.6)	0	0
Hypoaesthesia oral		12 (4.8)	7 (2.7)	1 (0.4)
Dry mouth		11 (4.4)	19 (7.5)	3 (1.2)
Nausea		7 (2.8)	6 (2.4)	4 (1.6)
Headache		6 (2.4)	12 (4.7)	12 (4.8)
Paraesthesia oral		6 (2.4)	7 (2.7)	1 (0.4)

All subjects self-administered the sublingual film

Treatment Emergent Adverse Events (TEAEs) with >2% incidence rate in one or more treatment groups are included, sorted by decreasing frequency in the order of 180 ug BXCL501, 120 ug BXCL501, Placebo. Subjects counted once at highest severity within each term based on MedDRA (Medical Dictionary for Regulatory Activities) version 23.0

Commercial Planning

Millions of Patients Experience Agitation Across Neuropsychiatric Diseases

Schizophrenia, Bipolar Disorder

Total Disease Prevalence

~65MM

~8MM

~40% agitation

High Unmet Medical Need

Frequent Episodes per Year

Significant Financial Burden

Sources: WHO Mental Disorders, 29 November 2019, Company Estimates

Key Activities Over the Next 12 Months

Building Cross-Functional Team

- Core Team: Q3 ‘20
- Medical: MSL team of 20, including Payer MSLs, launching in Q1 ‘21
- Market Access: Account Management Team on board mid ‘21
- Sales: 75-100 representative sales force to cover high volume institutions

Commercialization Outside U.S.

- Seeking regional partners for Japan and Europe

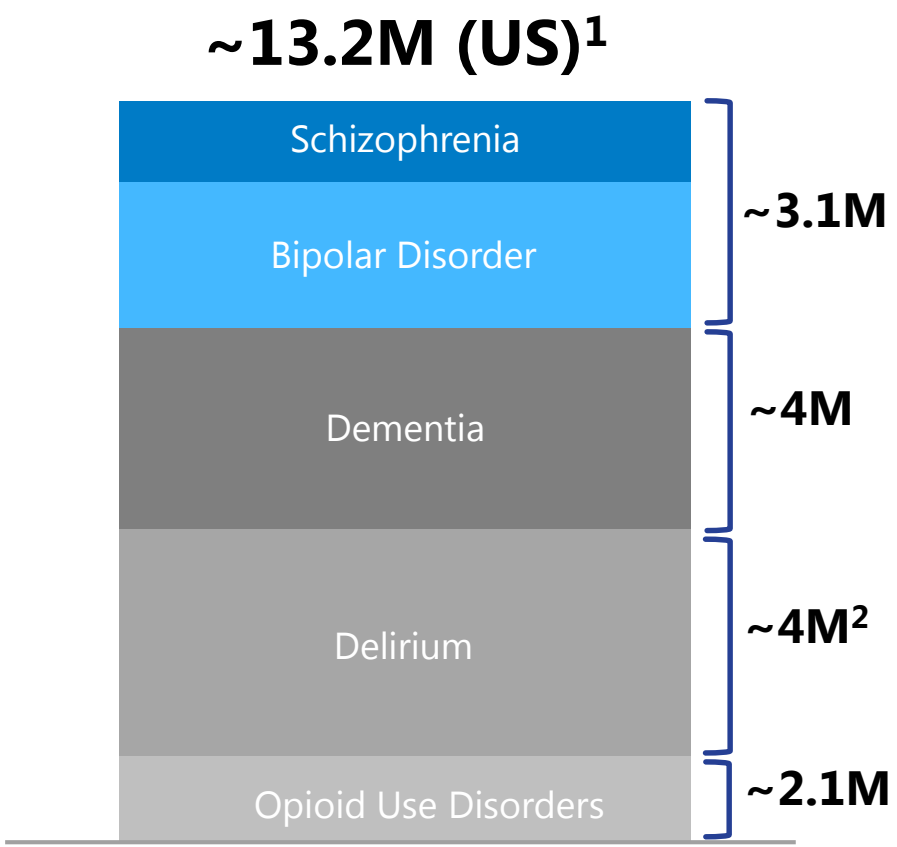
Conclusion and What's Ahead

Conclusion

- ✓ BXCL501 achieved all efficacy endpoints: primary, secondary and exploratory
  - Clinically meaningful improvement
  - Rapid onset of action and durable responses
  - Well tolerated with no serious adverse events
- ✓ NDA submission to U.S. FDA planned for Q1 2021
- ✓ BXCL501's mechanism of action in patients with agitation appeared to be independent of underlying neuropsychiatric conditions

Expanding BXCL501 Commercial Opportunities

Indication	Upcoming milestones
Schizophrenia & Bipolar Disorder	✓ SERENITY I & II topline results • NDA submission expected Q1 2021
Dementia	✓ TRANQUILITY Phase 1b/2 trial initiated • Topline readout expected mid-2020
Opioid Withdrawal Symptoms	✓ RELEASE Phase 1b/2 trial initiated • Topline readout expected Q1 2021
Delirium	✓ Compassionate use program at MGH • Clinical planning
PTSD, traumatic brain injury, alcohol withdrawal and treatment of phobias	• Evaluating commercial opportunity



1. Internal company estimates based on analysis of primary market research, prescription database, and published data.
2. Includes patients with agitated delirium in ICU, medical and surgical wards.

Q&A

Thank You!

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