Phase 1b/2 TRANQUILITY Trial – Topline Results

Acute Treatment of Agitation in Patients with Dementia

January 5, 2021

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Agenda

Topic	Presenter
Opening Remarks	Vimal Mehta, Ph.D., CEO & Founder
Overview & Summary	Reina Benabou, M.D., Ph.D., Sr. VP, CDO
TRANQUILITY Trial Design & Results	Robert Risinger, M.D., VP, Clinical Development
Conclusion & What's Ahead	Vimal Mehta, Ph.D., CEO & Founder
Q&A	BioXcel Therapeutics Team

Agitation: Cause of Patient Distress & Caregiver Burden

Significant medical need with no FDA-approved treatments

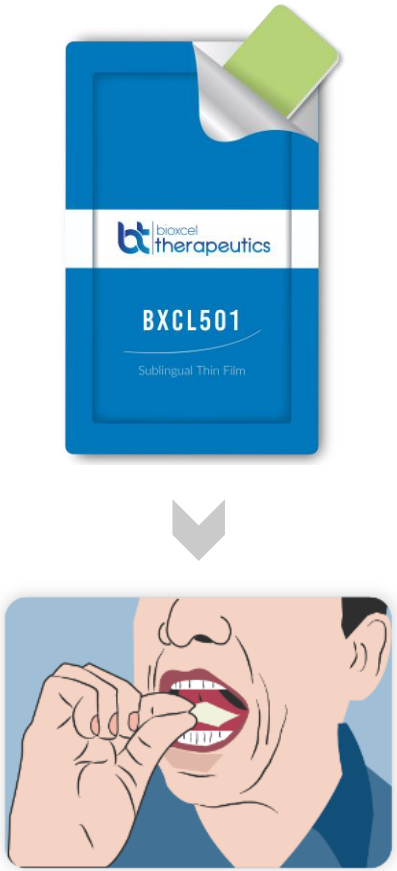
- Agitation is a common and difficult to manage symptom
- Dementia prevalence over 50M worldwide, with~6M in the U.S.
  - Up to 80% have Alzheimer's Disease
  - Up to 70% of patients experience agitation
  - In U.S., approximately 100M agitation episodes per year*
- Characterized by restless behaviour, improper physical and verbal actions, resulting in:
  - Endangerment to patients and others
  - Caregiver burden and burnout
  - Early Institutionalization and frequent ED visits
- No FDA-approved therapies and off-label therapies have black box warnings for the elderly
- BXCL501 has novel mechanism and highly differentiated approach

*Internal company estimate based on market research

Significant Improvement in Agitation Associated With Dementia

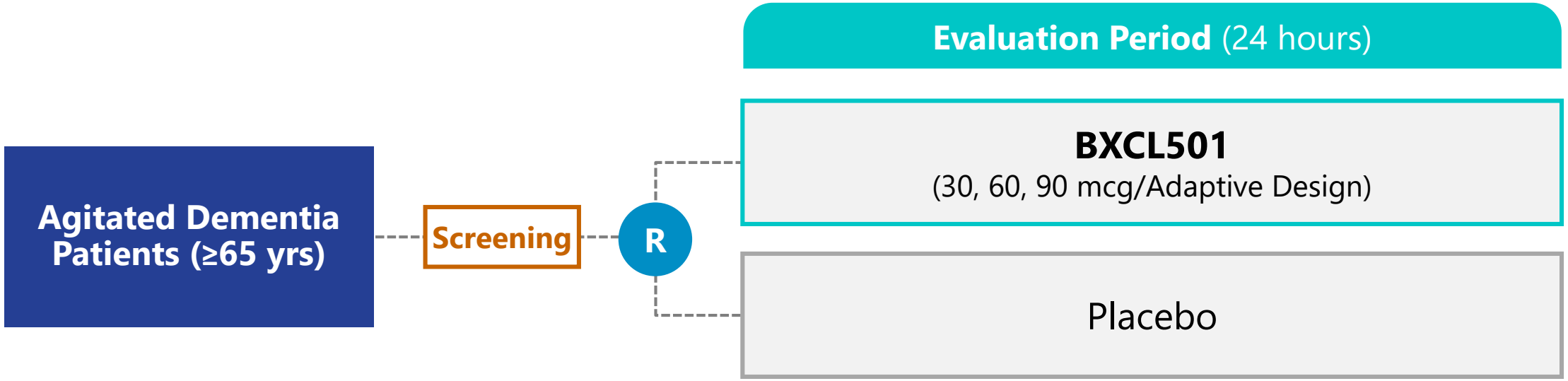
- ✓ BXCL501 was well tolerated with no severe or serious adverse events
  - ✓ No cases of syncope or falls
- ✓ Statistically significant reductions in agitation achieved at 2 hours post-dose with 60 mcg cohort as measured by the PEC, PAS and Mod-CMAI scales, with:
  - ✓ Numerical separation as early as 30 min in PEC score, with statistically significant reductions from baseline observed at 60 min in PEC & PAS scores
  - ✓ Duration of response lasted 8 hrs after treatment
  - ✓ All exploratory endpoints demonstrated statistically significant reductions from baseline in agitation
- ✓ The 30 mcg dose cohort showed numerical improvements across all scales
- ✓ Higher exposure levels observed in elderly dementia patients potentially enable efficacy at lower doses
- ✓ Results provide a clear path to a pivotal program for BXCL501 in dementia

TRANQUILITY Trial Design



TRANQUILITY: Phase 1b/2 Proof-of-Concept Trial in Dementia

Goal is to Identify Tolerable and Effective Dose(s) for Late-Stage Trial



Primary Endpoints: Safety & Tolerability
Secondary Endpoints: Magnitude of Calming Effect Using PAS, PEC and Modified CMAI

Inclusion/Exclusion Criteria

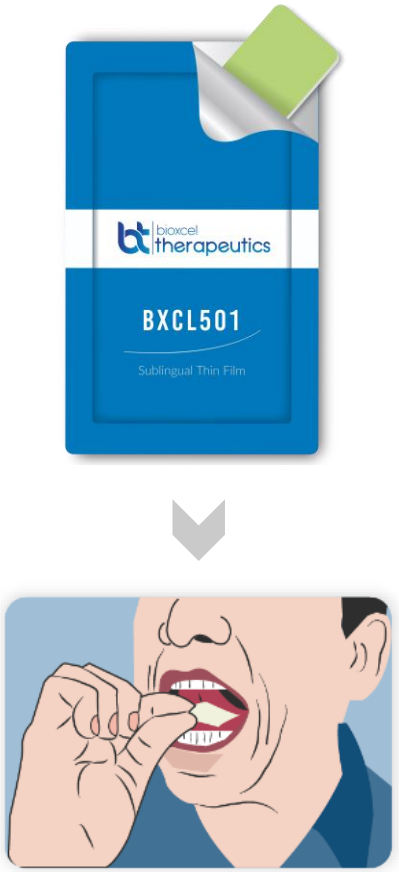
Inclusion Criteria:

- Diagnosis of dementia using DSM-5 criteria
- History of acute agitation that impairs social activities, requires staffing, medical intervention, or impairs daily living
- Total score of ≥8 on the 4 items comprising the PAS at screening and baseline
- Score of ≥2 on at least 1 of the 4 items on the PAS at baseline

Exclusion Criteria:

- Agitation caused by acute intoxication or positive identification of non-prescription drugs during urine screening
- Use of benzodiazepines, other sedatives, hypnotics, or antipsychotics 4 hours before study treatment
- Treatment with alpha-1 noradrenergic blockers or alpha adrenergic antagonists within 8 hours prior to dosing

Safety, Tolerability and Efficacy Results



Demographics and Baseline Characteristics

	BXCL501 30 mcg (N=16)	**BXCL501** 60 mcg (N=20)	Placebo (N=14)	Overall (N=54*)
Mean age (SD)	75.7 (8.0)	77.8 (6.3)	75.9 (9.0)	76.0 (7.8)
Female (%)	5 (31.3)	10 (50.0)	8 (57.1)	23 (42.6)
Race (% white/non-white)	81.3/18.7	70.0/30.0	92.9/7.1	75.9/24.1
BMI	27.4 (5.7)	23.6 (3.8)	25.2 (6.9)	25.3 (5.4)
Diagnosis (n/%)				
AD	14 (87.5)	17 (85.0)	12 (85.7)	47 (87.0)
Vascular	1 (6.3)	2 (10)	1 (1.7)	4 (7.4)
Frontotemporal Dementia	1 (6.3)	1 (5.0)	0	2 (3.7)
Unknown	0	0	1 (7.1)	1 (1.9)
PEC baseline (SD)	18.4 (1.5)	16.6 (3.5)	16.6 (2.7)	
PAS	8.9 (0.9)	9.1 (1.3)	8.7 (0.9)	

* 4 patients included from 90 mcg dose cohort

No discontinuations; All randomized patients completed trial

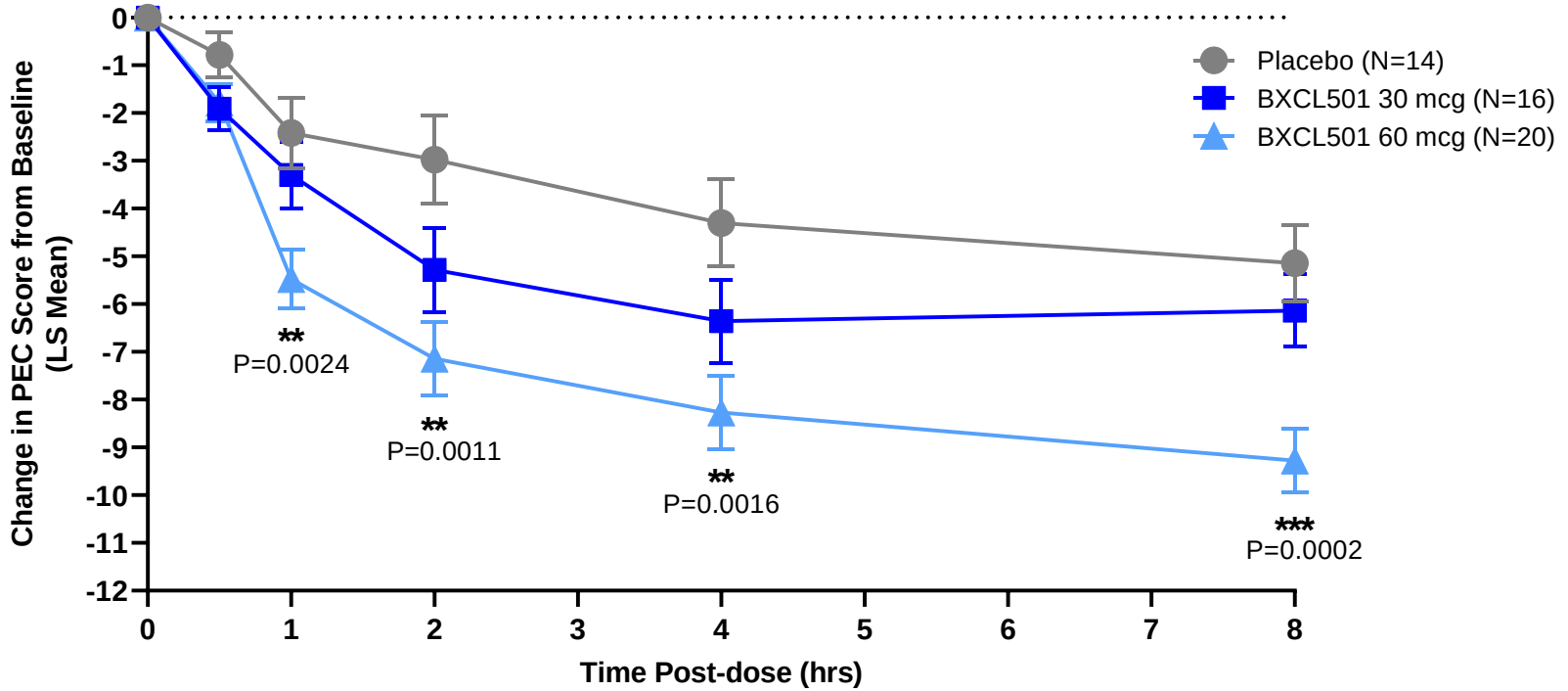
BXCL501 Well Tolerated with No Severe or Serious Adverse Events

		BXCL501 30 mcg (N=16)	BXCL501 60 mcg (N=20)	Placebo (N=14)
Somnolence*	Mild	8 (50.0 %)	11 (55.0 %)	1 (7.1 %)
	Moderate	0	1 (5.0 %)	
Hypotension	Mild	0 (0)	1 (5 %)	0
	Moderate	0 (0)	1 (5 %)	0
Orthostatic hypotension	Mild	0 (0)	1 (5 %)	0
	Moderate	1 (6.3 %)	0 (0)	0
Dizziness	Mild	1 (6.3 %)	1 (5 %)	0
	Moderate	0 (0)	0 (0)	
Bradycardia		0	1 (5 %)	0
Dry mouth		0	1 (5 %)	0
Nausea		0	1 (5 %)	0
Headache		0	1 (5 %)	0

*Verbatim; drowsy or feeling sleepy

All subjects self-administered the sublingual film

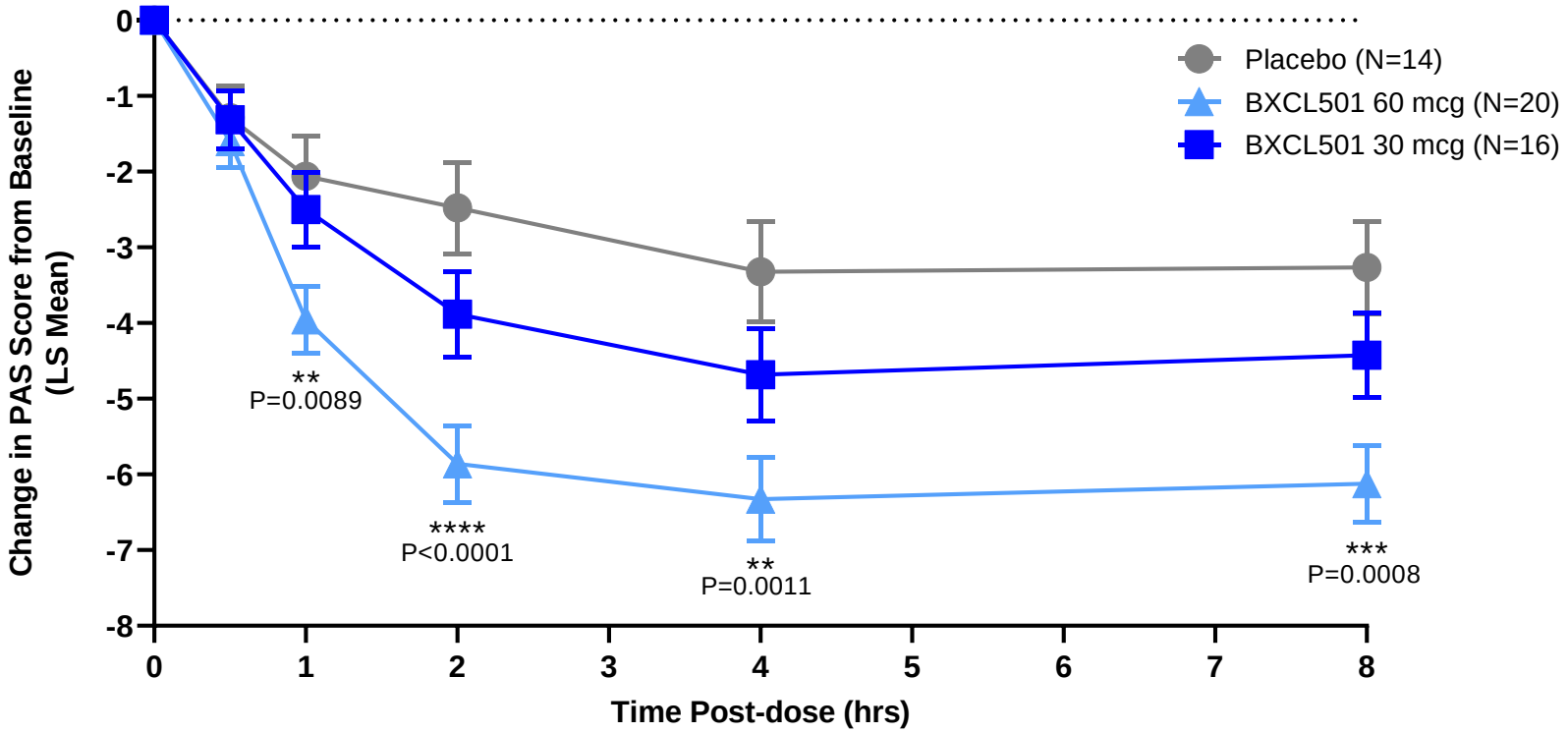
Rapid and Durable Response Demonstrated by PEC



Efficacy Results at 120 mins	PEC Total Score	Placebo	BXCL501 30 mcg	BXCL501 60 mcg
	Change from Baseline (LS Mean)	-2.9	-5.4	-7.1 **
	Response °	7%	25%	70% **

PANSS-Excitatory Component (PEC) is a 5 items scale: Excitement, Hostility, Tension, Uncooperativeness, Poor Impulse Control, rated 1-Absent to 7-Extreme
ITT analysis, Least Square Means ± SEM
° Proportion achieving ≥ 40% PEC reduction

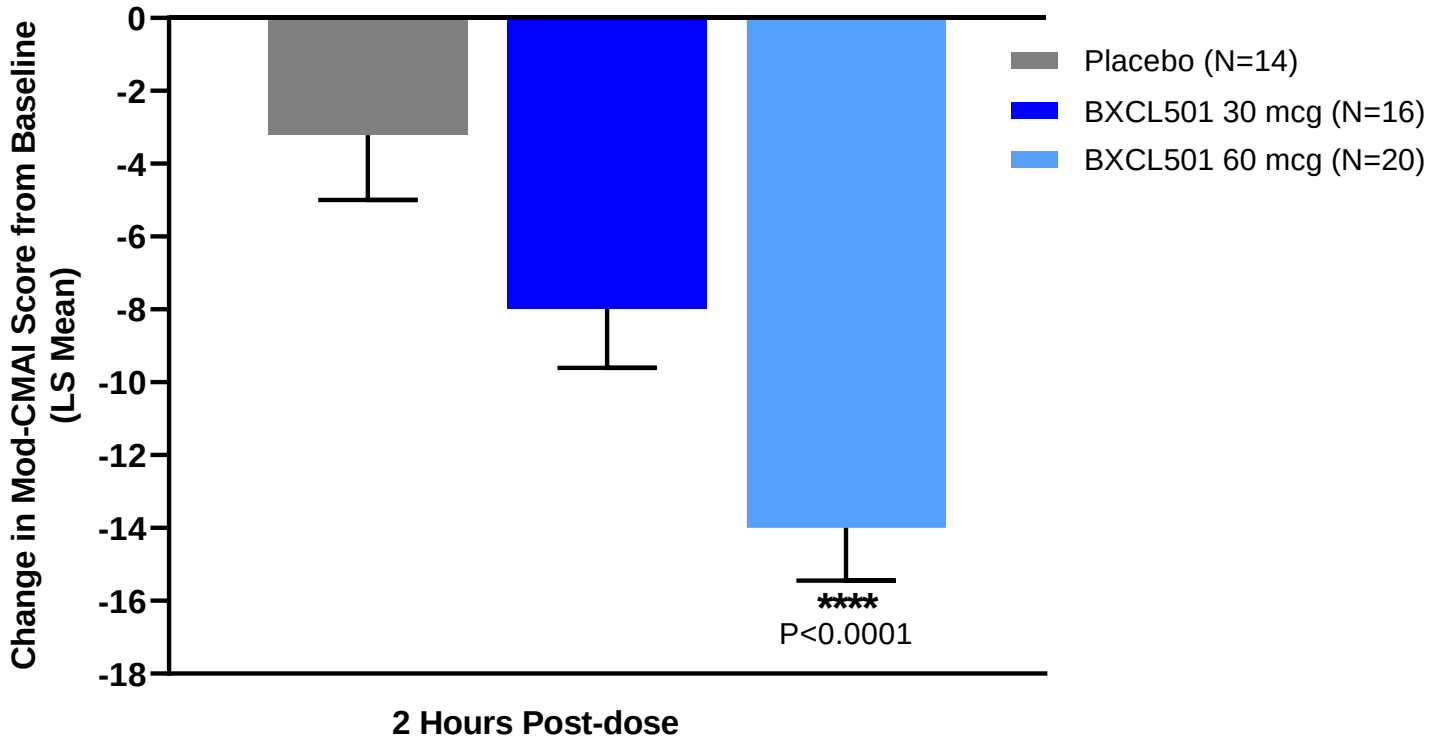
Rapid and Durable Response Confirmed by PAS



Efficacy Results at 120 mins	PAS Total Score	Placebo	BXCL501 30 mcg	BXCL501 60 mcg
	Change from Baseline (LS Mean)	-2.5	-3.9	-5.9 ****

Pittsburgh Agitation Scale (PAS) measures 4 behavior groups: aberrant vocalization, motor agitation, aggressiveness, and resisting to care rated 0- no agitation present to 4 – highest form of agitation.
ITT analysis, Least Square Means ± SEM

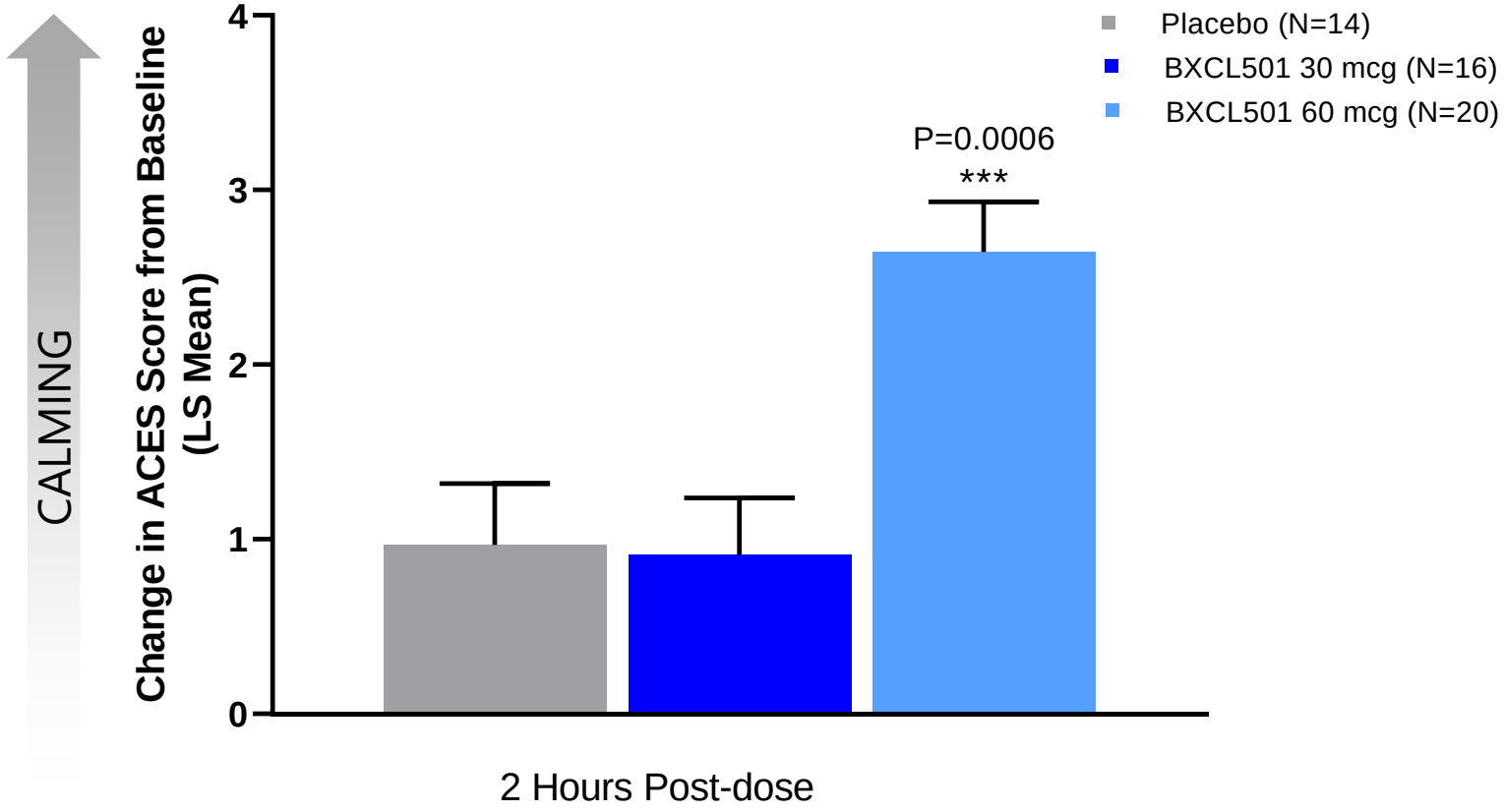
Rapid and Durable Response Validated Using Modified CMAI



Efficacy Results at 120 mins	Mod-CMAI Total Score	Placebo	BXCL501 30 mcg	BXCL501 60 mcg
	Change from Baseline (LS Mean)	**-3.2**	**-8.0**	**-14.0 ******

Modified Cohen-Mansfield Agitation (Mod-CMAI) is an inventory consisting of 29 behaviors, each rated on a 7-point scale of frequency: 1 – never to 7 – several times an hour. Only behaviors manifested by the subject at baseline were assessed throughout the study.
ITT analysis, Least Square Means ± SEM

Independent Confirmation of Calming by ACES

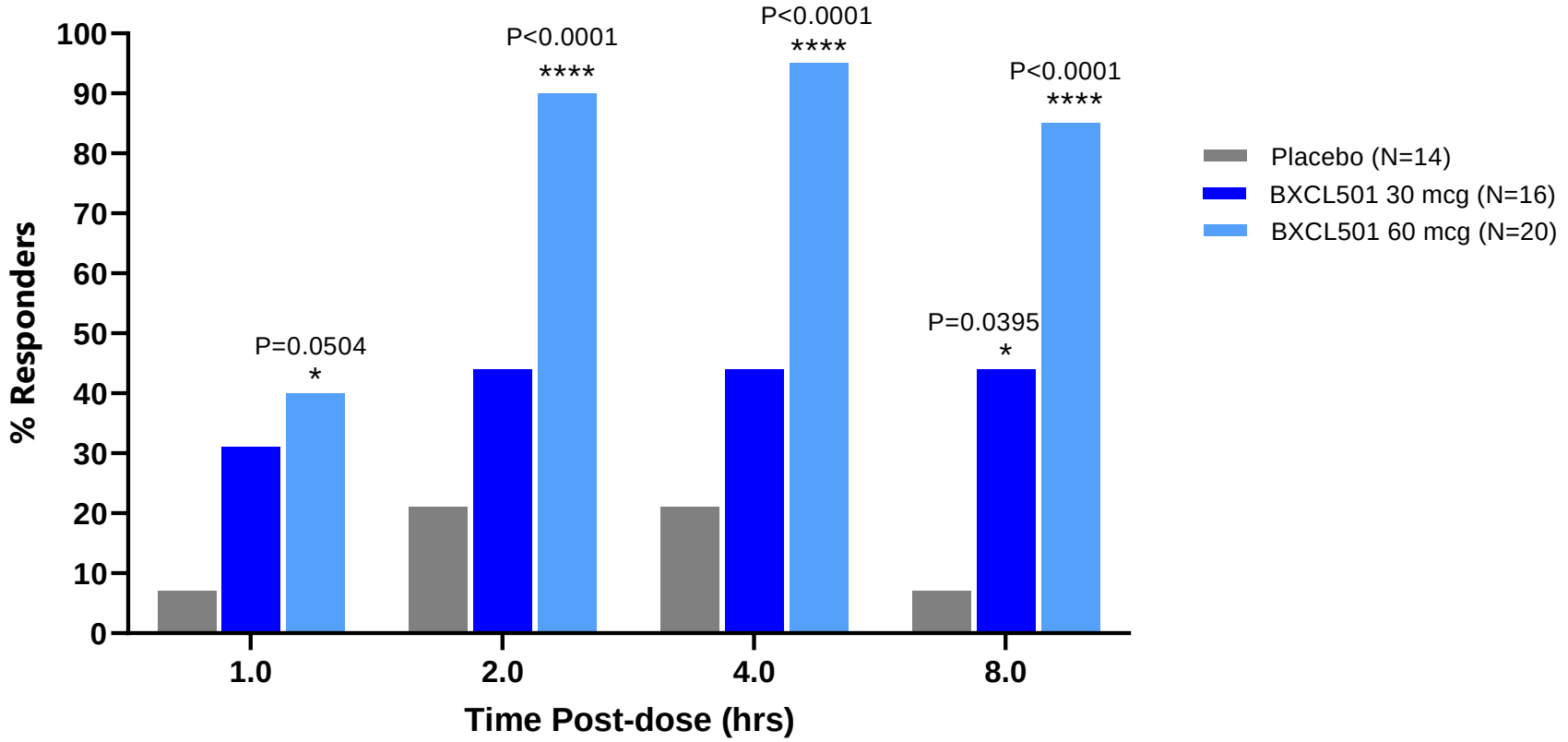


Significant calming observed at 60 mcg dose

The ACES consists of a single item that rates overall agitation and calming 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

Clinically Meaningful Improvement Confirmed by CGI-I

Responder rate of 90% at two hours after dosing



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

Conclusion and What's Ahead

Conclusion

- ✓ BXCL501 at the 60 mcg dose achieved all efficacy endpoints: primary, secondary and exploratory
  - Rapid onset of action and durable responses
  - Clinically meaningful improvement observed in agitation measures
  - Well tolerated with no severe or serious adverse events
- ✓ 30 mcg dose cohort showed numerical improvements across all scales
- ✓ Results provide a clear path to a pivotal program for BXCL501 in dementia
- ✓ TRANQUILITY results provide a strong foundation for our broad dementia development strategy, exploring acute to chronic agitation

BXCL501's Planned Development Across the Agitation Spectrum in Dementia

Agitation Spectrum	Pre-Agitation	Acute/Episodic Agitation	Sub-chronic Agitation	Chronic Agitation
BXCL501	Wearable + PRN	**PRN**	**PRN**	Daily (BXCL501 single or combination use)

Hospitals/EDs

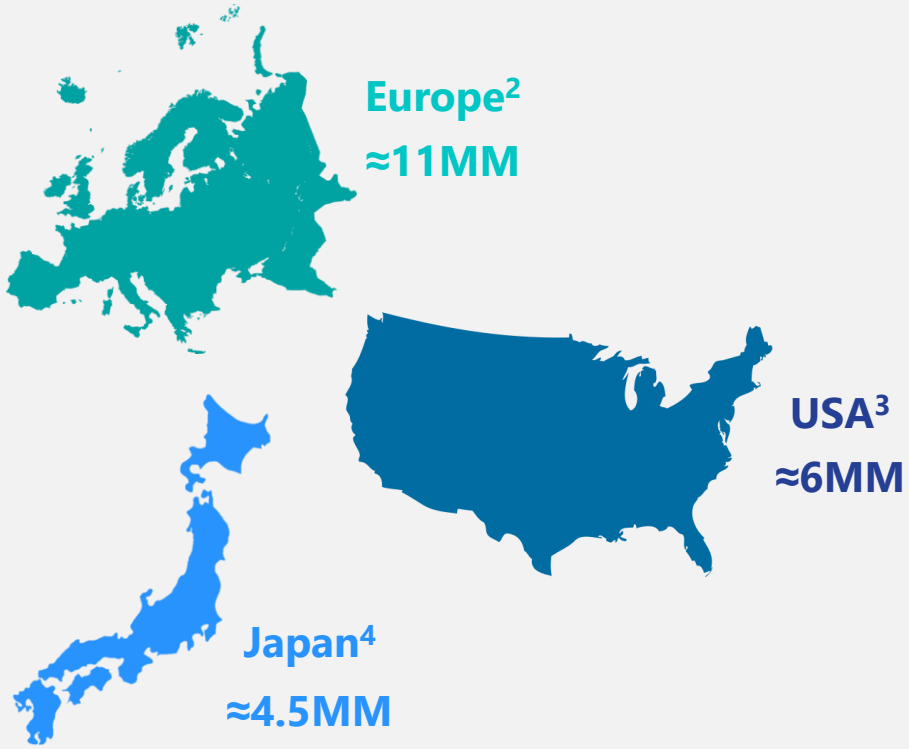
Assisted Living/Nursing Homes

Community/At Home

Commercial Opportunity in Dementia

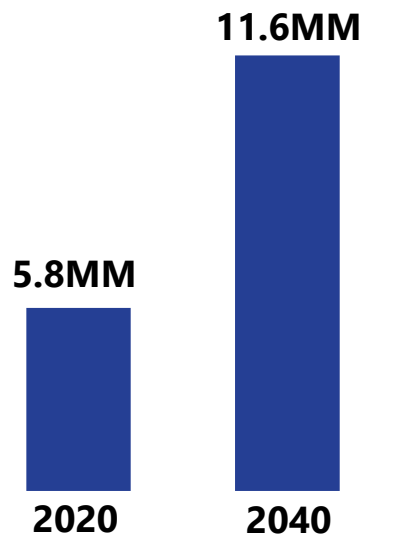
Highly prevalent global condition, with incidence increasing rapidly

2020 Dementia Global Prevalence ≈ 50MM[1]



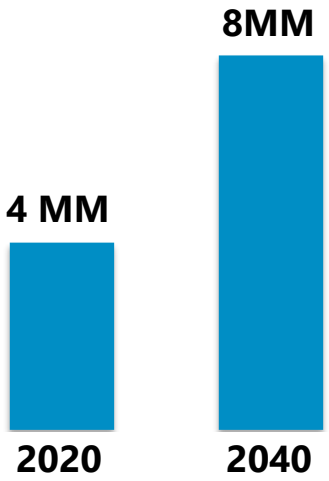
>40% of population in priority regions/countries

American 65+ with Alzheimer's Disease[2] to double by 2040[3]



Estimated Number of U.S. Patients with Agitation

Up to 70%
Experience agitation[5]



Approximately 100MM agitation episodes per year in the U.S. [6]

Sources: [1]WHO 2020, [2]Alzheimer's Europe Yearbook 2019; [3]Alzheimer's Association, [4]Alz.org Japan;; [5]Tractenberg, R Neuropsychiatry Clin Neuroscience 14:1, Winter 2002; [6]Internal company estimate based on market research

Q&A

Thank You!

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