Phase 1b/2 TRANQUILITY Trial – Program Update

Acute Treatment of Dementia Related Agitation

NASDAQ: BTAI

March 3, 2021

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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

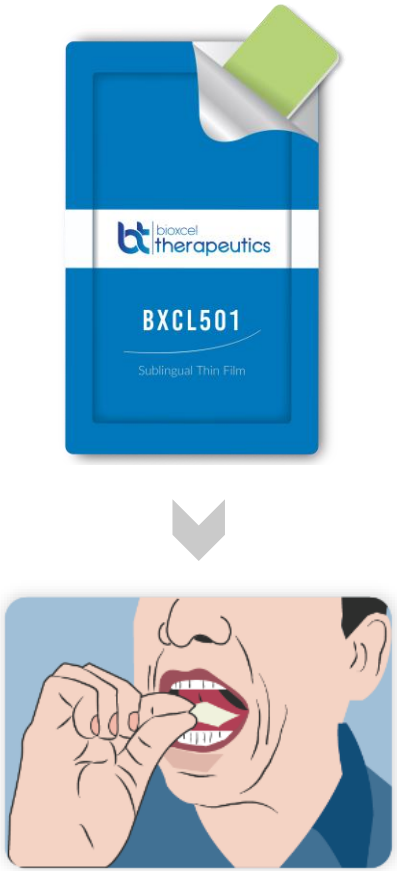
Dementia Related Agitation Program Update

- Review of TRANQUILITY data showed the 30 mcg dose met statistical significance at two hours as measured by PEC, PAS and CMAI
  - Two patients were mis-categorized within the 30 mcg cohort at the clinical site
- Initiated supplemental study to evaluate a 40 mcg dose to help inform clinical development strategy across dementia care settings
  - Additional insights generated will support clinical development strategy for all segments of the dementia market
- The end of Phase 2 meeting with the FDA has been scheduled for Q2 2021 to finalize study design, dosing and endpoints for registrational program
- Pivotal Phase 3 program expected to begin in the second half of 2021

Significant Improvement in Agitation Associated With Dementia

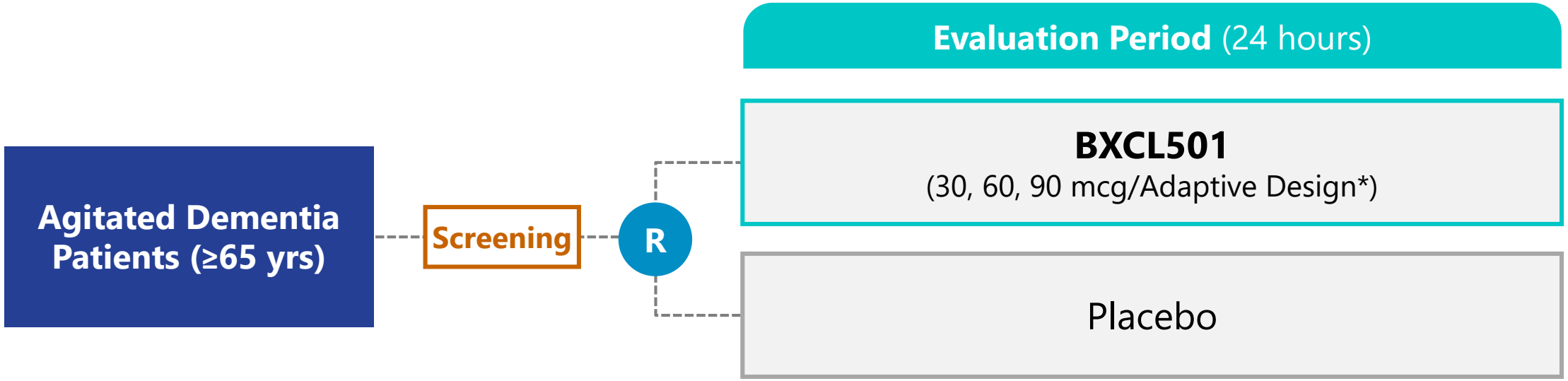
- BXCL501 was well tolerated with no severe or serious adverse events
    - No cases of syncope or falls
- Statistically significant reductions in agitation achieved at 2 hours post-dose with both 30 and 60 mcg cohort as measured by the PEC, PAS and Mod-CMAI scales, with:
    - Numerical separation as early as 30 min in PEC score, with statistically significant reductions from baseline observed at 60 min in PEC & PAS scores with 60 mcg dose
    - Duration of response lasted 8 hours after treatment with 60 mcg dose
    - All exploratory endpoints demonstrated statistically significant reductions from baseline in agitation with 60 mcg dose
- Higher exposure levels observed in elderly dementia patients enable efficacy at **lower doses;** will allow for testing of BXCL501 in the full range of treatment settings, from assisted living to home care
- 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

* A 40mcg dose cohort study of BXCL501 initiated

Inclusion/Exclusion Criteria

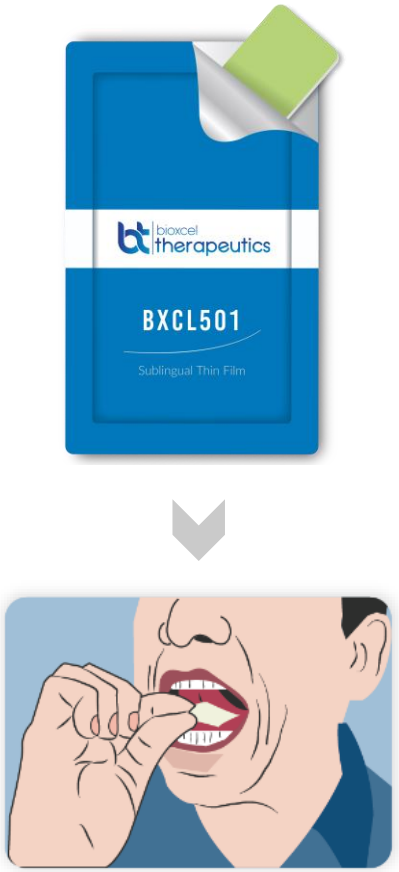
Inclusion Criteria

- 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)	75.8 (8.0)	77.8 (6.4)	75.9 (8.9)	76.0 (7.8)
Female (%)	5 (31.3)	10 (50.0)	8 (57.1)	23 (42.6)
Race (% white/non-white)	81.3/18.8	70.0/30.0	92.9/7.1	75.9/24.1
BMI	27.5 (5.7)	23.6 (3.8)	25.1 (7.0)	25.4 (5.4)
Diagnosis (n/%)				
AD	14 (87.5)	17 (85.0)	13 (92.9)	47 (87.0)
Vascular	1 (6.3)	2 (10)	0	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)	18.3 (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

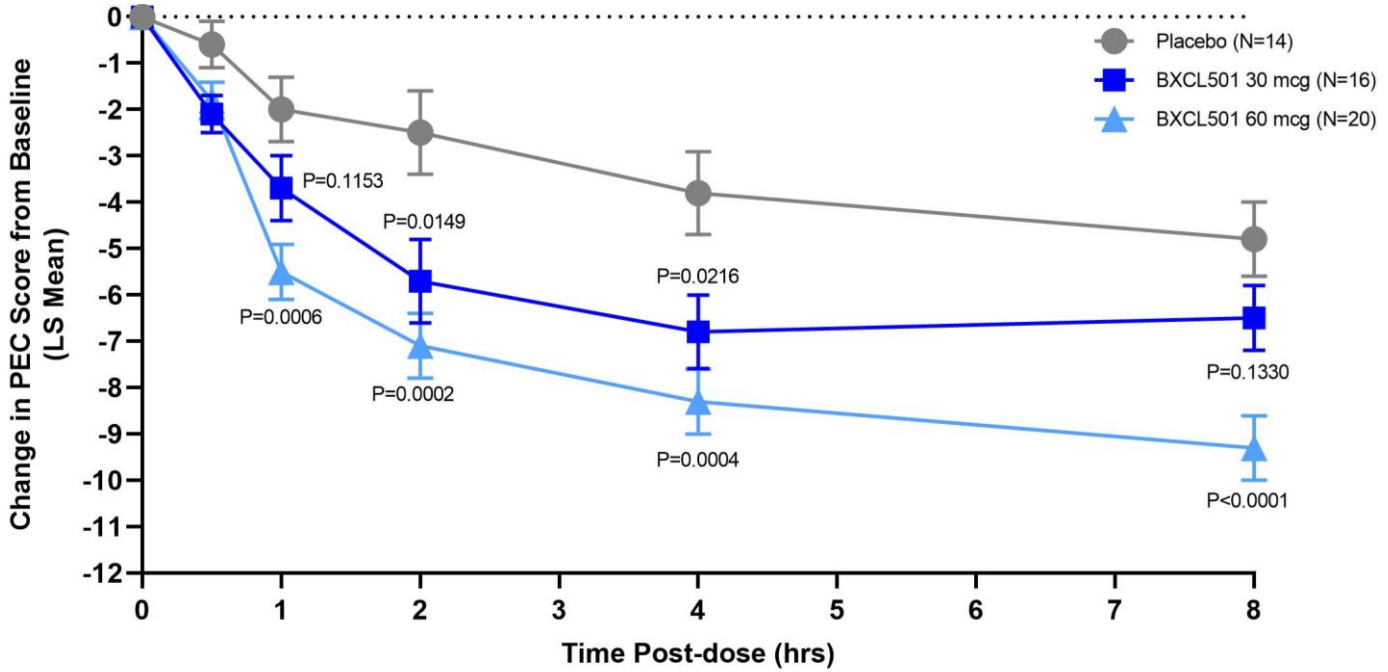
BXCL501 Well Tolerated with No Severe or Serious Adverse Events

		BXCL501 30 mcg (N=16)	BXCL501 60 mcg (N=20)	Placebo (N=14)
Somnolence*	Mild	9 (56.3%)	11 (55.0 %)	0
	Moderate	0	1 (5.0 %)	
Hypotension	Mild	0 (0)	1 (5.0 %)	0
	Moderate	0 (0)	1 (5.0 %)	0
Orthostatic hypotension	Mild	0 (0)	1 (5.0 %)	0
	Moderate	1 (6.3 %)	0 (0)	0
Dizziness	Mild	1 (6.3 %)	1 (5.0 %)	0
	Moderate	0 (0)	0 (0)	
Bradycardia		0	1 (5.0 %)	0
Dry mouth		0	1 (5.0 %)	0
Nausea		0	1 (5.0 %)	0
Headache		0	1 (5.0 %)	0

*Verbatim; drowsy or feeling sleepy

All subjects self-administered the sublingual film

Rapid and Durable Response Demonstrated by PEC

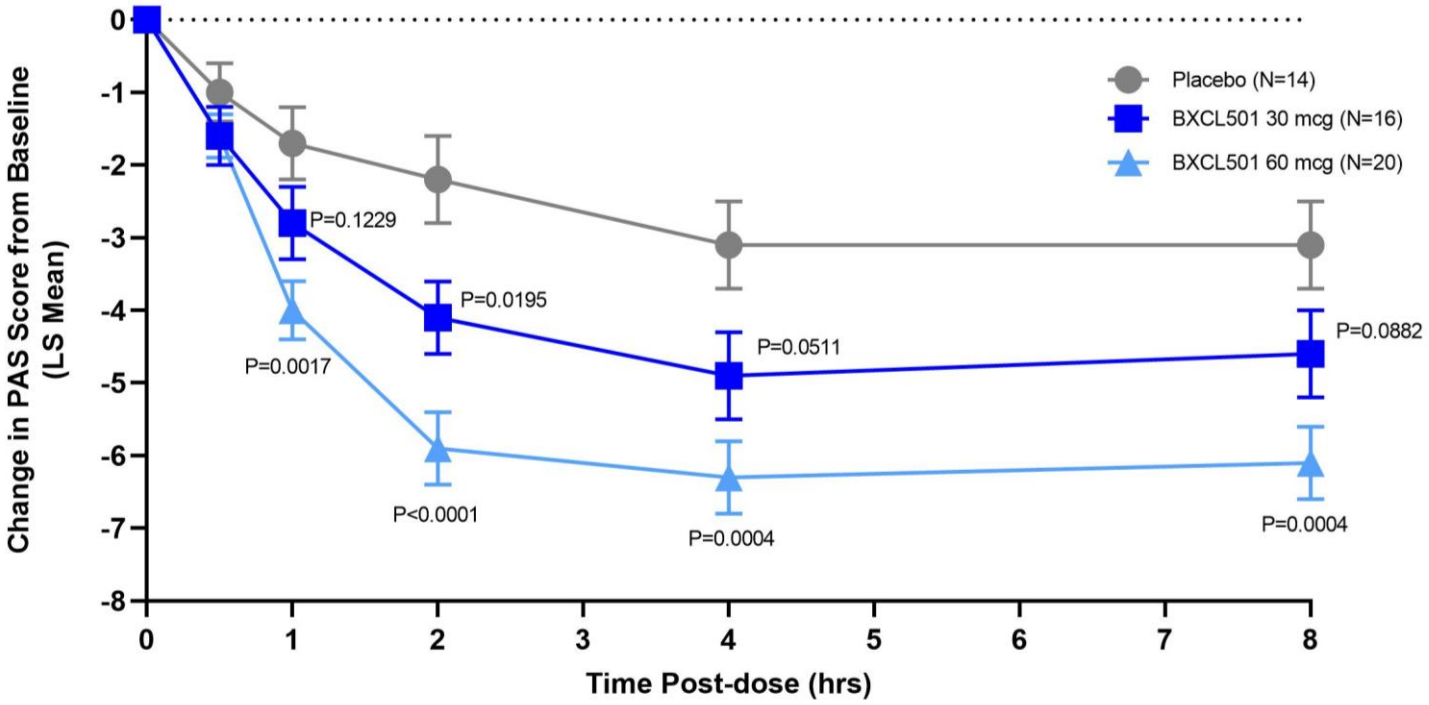


P values at 0.5 hrs are 0.0295 for BXCL501 30 mcg and 0.0568 for BXCL501 60 mcg

Efficacy Results at 120 mins	PEC Total Score	Placebo	BXCL501 30 mcg	BXCL501 60 mcg
	Change from Baseline (LS Mean)	-2.5	-5.7	-7.1
	Response °	0%	31%	70%

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

Rapid and Durable Response Confirmed by PAS

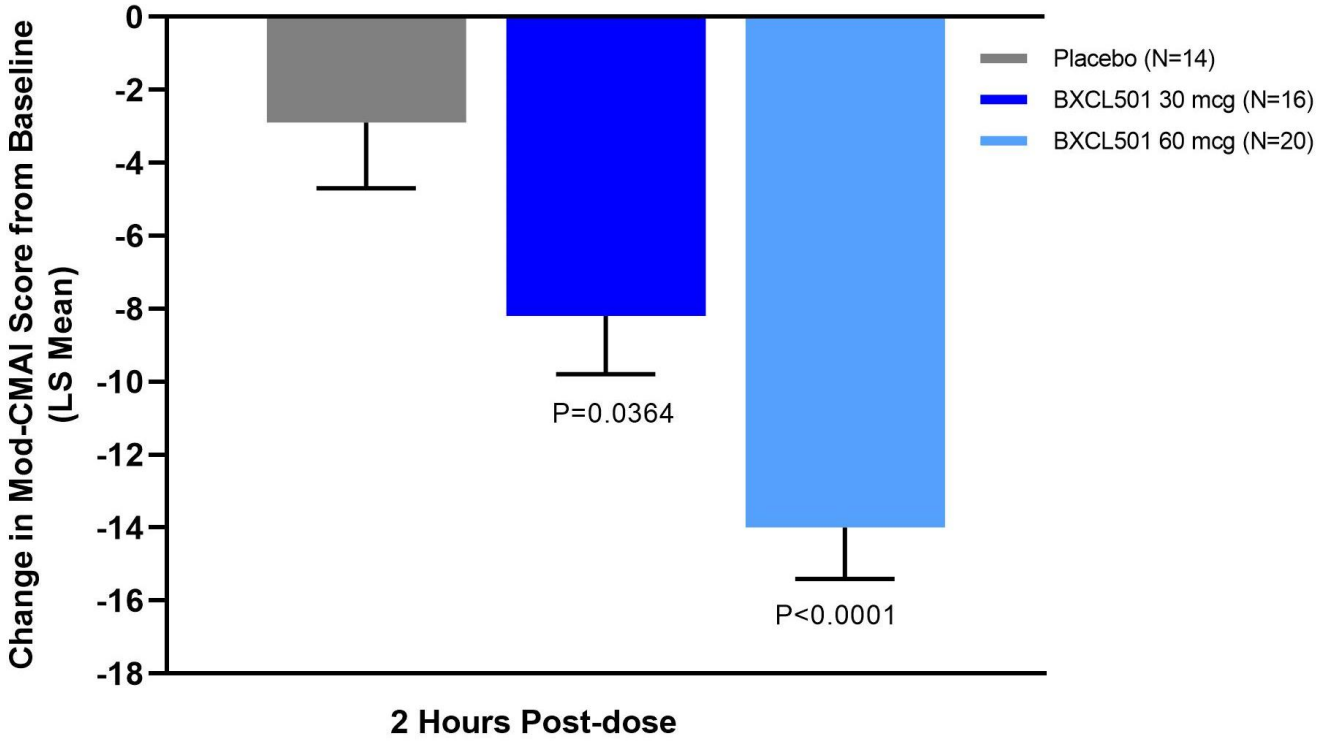


P values at 0.5 hrs are 0.3162 for BXCL501 30 mcg and 0.2631 for BXCL501 60 mcg

Efficacy Results at 120 mins	PAS Total Score	Placebo	BXCL501 30 mcg	BXCL501 60 mcg
	Change from Baseline (LS Mean)	-2.2	-4.1	-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.
As treated analysis, Least Square Means ± SEM

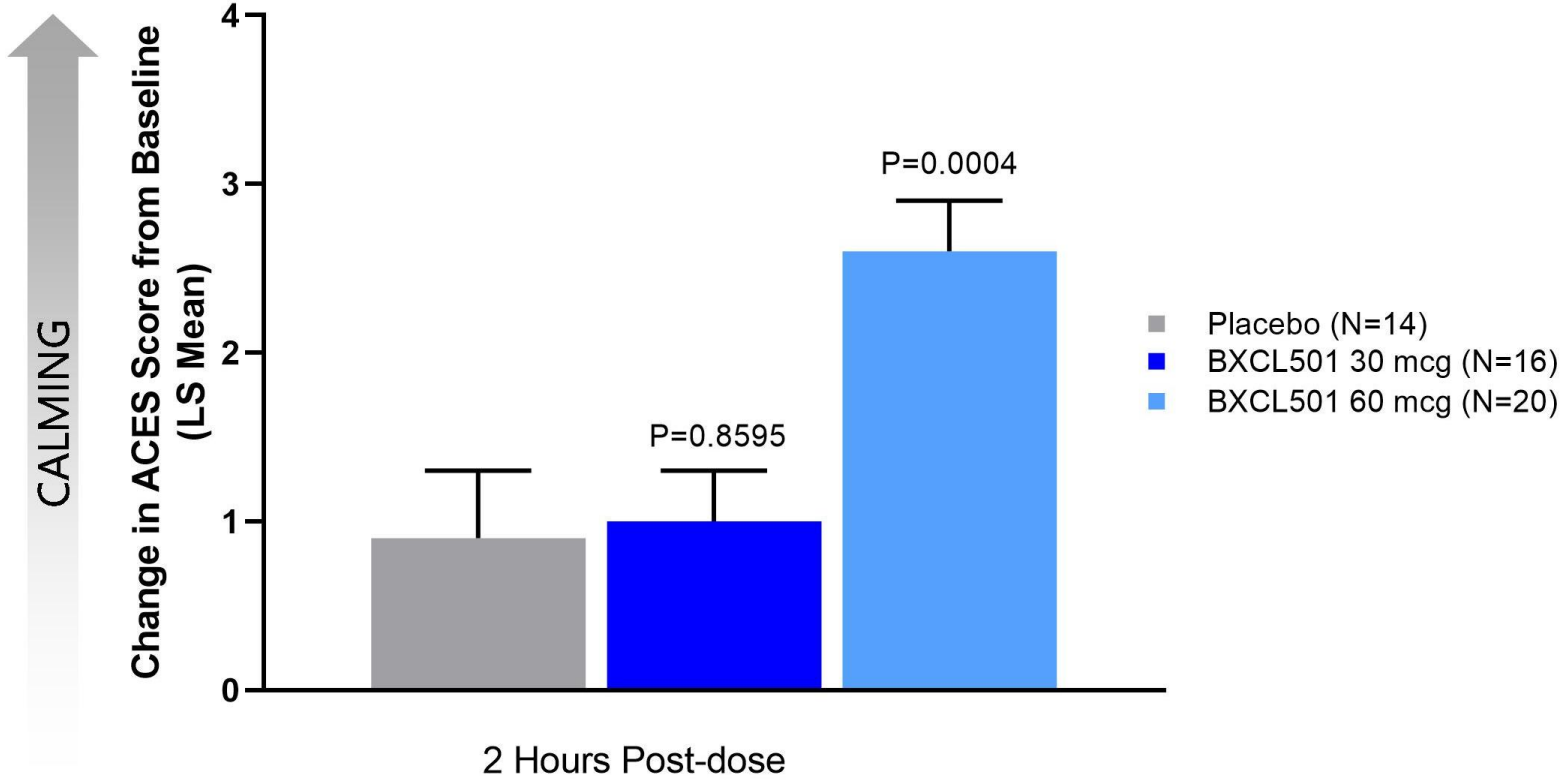
Rapid and Durable Response Validated Using Modified CMAI



Efficacy Results at 120 mins	Mod-CMAI Total Score	Placebo	BXCL501 30 mcg	BXCL501 60 mcg
	Change from Baseline (LS Mean)	**-2.9**	**-8.2**	**-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.
As treated analysis, Least Square Means ± SEM

Independent Confirmation of Calming by ACES

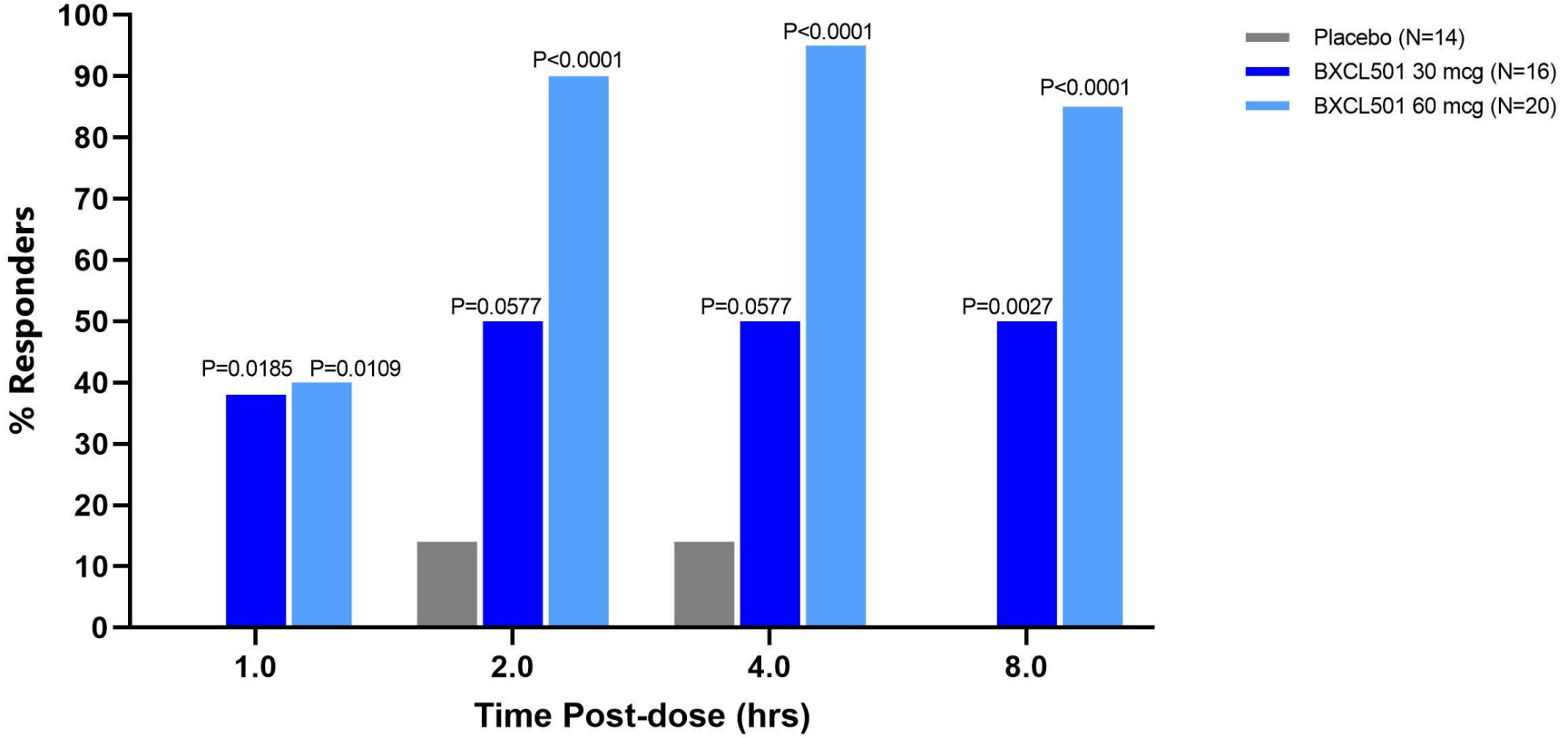


Significant calming observed at 60 mcg dose

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 for 60 mcg



No placebo responders (0%) at 1 hour post-dose and at 8 hours post-dose

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.
As treated analysis

Conclusion and What's Ahead

Conclusion

- ✓ BXCL501 at 30 mcg and 60 mcg met statistical significance across multiple efficacy endpoints:
  - Rapid onset of action and durable responses for at least 8 hours with 60 mcg dose
  - Clinically meaningful improvement in agitation at both doses
  - Well tolerated with no severe or serious adverse events at both doses
- ✓ Results provides a clear path to a pivotal program for BXCL501 in dementia
- ✓ TRANQUILITY results provide a strong foundation for our broad dementia development strategy, exploring for full range of dementia care settings for 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)

Treatment Settings Spectrum

Hospitals/EDs

Assisted Living/ Nursing Homes

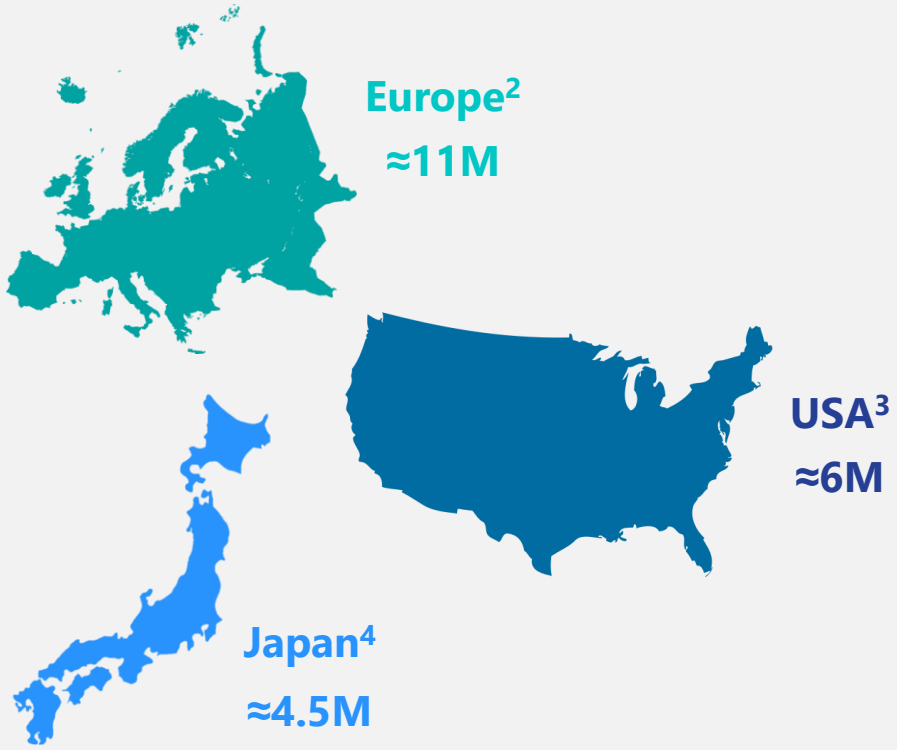
Community/At Home

*As needed

Commercial Opportunity in Dementia

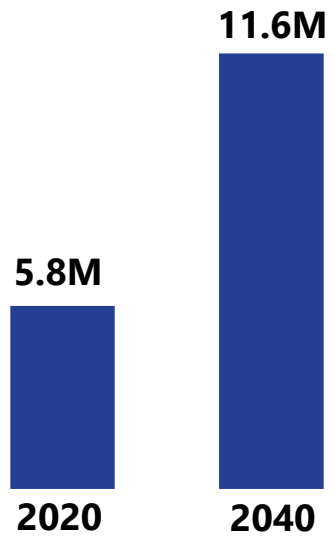
Highly prevalent global condition, with incidence increasing rapidly

2020 Dementia Global Prevalence ≈ 50M[1]



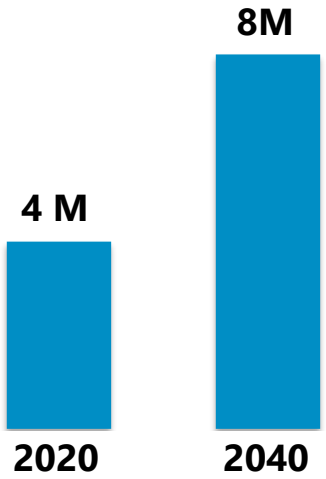
>40% of population in priority regions/countries

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



Up to 70%
Experience agitation[5]

Estimated Number of U.S. Patients with Agitation



Approximately 100M 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