BioXcel Therapeutics Announces First Patients Dosed in SERENITY III Phase 3 Trial for Acute Treatment of Agitation in Adults with Bipolar I or II Disorder or Schizophrenia

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SERENITY III will evaluate the efficacy and safety of BXCL501 for at-home use

Top-line pivotal data expected in 1H 2023

Estimated 23 million annual agitation episodes in the home-setting would more than double current market opportunity for BXCL501 in the U.S. [1-4]

NEW HAVEN, Conn., Nov. 30, 2022 (GLOBE NEWSWIRE) -- BioXcel Therapeutics, Inc. (Nasdaq: BTAI), a biopharmaceutical company utilizing artificial intelligence approaches to develop transformative medicines in neuroscience and immuno-oncology, today announced that the first 13 patients have been dosed in Part 1 of the pivotal Phase 3 SERENITY III trial investigating at-home use of BXCL501 (dexmedetomidine) sublingual film, the Company's proprietary, orally dissolving film, for agitation associated with bipolar I or II disorder or schizophrenia.

"Evaluating BXCL501 for at-home use is an exciting and important milestone that potentially expands the market opportunity for the treatment of agitation and drives the growth of our neuroscience franchise," said Robert Risinger, M.D., Chief Medical Officer, Neuroscience of BioXcel Therapeutics. "We anticipate top-line data from SERENITY III as well as our TRANQUILITY II trial for Alzheimer's-related agitation in the first half of 2023. These two near-term pivotal data readouts further reinforce the potential of BXCL501 to address the unmet medical needs of millions of patients."

The treatment of agitation represents a significant market opportunity. There are approximately 39 million reported agitation episodes that occur in the U.S. each year related to bipolar disorders and schizophrenia.[1-4] 23 million of these episodes occur outside of the institutional setting, potentially more than doubling the current market opportunity for BXCL501.

SERENITY III is a two-part, double-blinded, placebo-controlled pivotal study designed to evaluate BXCL501 60mcg dose for at-home use. This strategic trial design follows a Type B meeting with the U.S. Food and Drug Administration and observed dose-dependent responses in a prior Phase 1/2b study assessing a range of doses. The first part of the study is similar to SERENITY I and II and designed to assess the efficacy and safety of a 60mcg dose in acutely agitated patients with bipolar disorder or schizophrenia in a monitored setting. The primary efficacy endpoint is the change from baseline in Positive and Negative Syndrome Scale-Excitatory Component (PEC) total score at two hours after dosing compared to placebo. In Part 1, approximately 200 patients will be enrolled at up to approximately 20 clinical sites in the U.S. The second part of the study is designed to assess the safety of 60mcg dose compared to placebo when self-administered at home. SERENITY III will utilize many of the same investigators and clinical sites as SERENITY I and II.

About BXCL501
BXCL501 is a proprietary, orally dissolving film formulation of dexmedetomidine, a selective alpha-2 adrenergic receptor agonist. BioXcel Therapeutics believes that BXCL501 potentially targets an important mediator of agitation, and the Company has observed anti-agitation results in multiple clinical studies across several neuropsychiatric disorders. BXCL501 is under investigation for the acute treatment of agitation associated with bipolar I or II disorder or schizophrenia in the at-home setting, for the acute treatment of Alzheimer's-related agitation, and as an adjunctive treatment for major depressive disorder. The safety and efficacy of BXCL501 for these investigational uses have not been established. BXCL501 has been granted Breakthrough Therapy designation for the acute treatment of agitation associated with dementia and Fast Track designation for the acute treatment of agitation associated with schizophrenia, bipolar disorders, and dementia.

About Agitation Associated with Bipolar Disorders and Schizophrenia
Agitation is a common and difficult-to-manage symptom associated with bipolar I or II disorder or schizophrenia. Early identification and prompt intervention to relieve agitation are essential to avoid symptomatic escalation and the emergence of aggression. Expert consensus best-practice guidelines have recommended that agitation should be treated by a combination of behavioral calming techniques, verbal de-escalation, and medications that are voluntarily accepted by patients without coercion. The goal of using medication is to calm the patient so that he or she can be more accurately assessed by clinicians. Medication used in this manner is consistent with current guidelines, which state that the proper endpoint of medication administration is calming without inducing sleep. The Company believes this approach may help avoid the costly and traumatic use of coercive techniques like physical restraint and seclusion, which may result in admission and prolonged hospitalization.[5]

About BioXcel Therapeutics, Inc.
BioXcel Therapeutics, Inc. is a biopharmaceutical company utilizing artificial intelligence approaches to develop transformative medicines in neuroscience and immuno-oncology. The Company's drug re-innovation approach leverages existing approved drugs and/or clinically validated product candidates together with big data and proprietary machine learning algorithms to identify new therapeutic indices. The Company's commercial product, IGALMI™ (developed as BXCL501), is a proprietary, sublingual film formulation of dexmedetomidine approved for the acute treatment of agitation associated with schizophrenia or bipolar I or II disorder in adults. The safety and effectiveness of IGALMI has not been established beyond 24 hours from the first dose. For more information, please visit IGALMIhcp.com and also see the IGALMI full Prescribing Information. BXCL501 is under evaluation for at-home use for the acute treatment of agitation in bipolar and schizophrenia patients, for acute treatment of Alzheimer's-related agitation, and as an adjunctive treatment for major depressive disorder. The safety and efficacy of BXCL501 for these uses have not been established. The Company is also developing BXCL502 as a potential therapy for chronic agitation in dementia. Under its subsidiary, OnkosXcel Therapeutics, the Company is developing BXCL701, an investigational, orally administered, systemic innate immune activator for the treatment of aggressive forms of prostate cancer. The safety and efficacy of BXCL502 and BXCL701 have not been established. For more information, please

visit bioxceltherapeutics.com.

Forward-Looking Statements

This press release includes “forward-looking statements” within the meaning of the Private Securities Litigation Reform Act of 1995. We intend such forward-looking statements to be covered by the safe harbor provisions for forward-looking statements contained in Section 27A of the Securities Act of 1933, as amended (the “Securities Act”) and Section 21E of the Securities Exchange Act of 1934, as amended (the “Exchange Act”). All statements contained in this press release other than statements of historical fact should be considered forward-looking statements, including, without limitation, the Company’s expected timing of, trial design and data results from, clinical trials of BXCL501. When used herein, words including “anticipate,” “believe,” “can,” “continue,” “could,” “designed,” “estimate,” “expect,” “forecast,” “goal,” “intend,” “may,” “might,” “plan,” “possible,” “potential,” “predict,” “project,” “should,” “target,” “will,” “would” and similar expressions are intended to identify forward-looking statements, though not all forward-looking statements use these words or expressions. 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 the Company’s current expectations and various assumptions. The Company believes there is a reasonable basis for its expectations and beliefs, but they are inherently uncertain. The Company may not realize its expectations, and its beliefs may not prove correct. Actual results could differ materially from those described or implied by such forward-looking statements as a result of various important factors, including, without limitation, its limited experience in drug discovery and drug development; its dependence on the success and commercialization of IGALMI™, BXCL501, BXCL502 and BXCL701 and other product candidates; its lack of experience in marketing and selling drug products; the failure of preliminary data from its clinical studies to predict final study results; failure of its early clinical studies or preclinical studies to predict future clinical studies; its ability to receive regulatory approval for its product candidates; its ability to enroll patients in its clinical trials; undesirable side effects caused by the Company’s product candidates; and the other important factors discussed under the caption “Risk Factors” in its Quarterly Report on Form 10-Q for the quarterly period ended September 30, 2022, as such factors may be updated from time to time in its other filings with the SEC, which are accessible on the SEC’s website at www.sec.gov. These and other important factors could cause actual results to differ materially from those indicated by the forward-looking statements made in this press release. Any such forward-looking statements represent management’s estimates as of the date of this press release. While the Company may elect to update such forward-looking statements at some point in the future, except as required by law, it disclaims any obligation to do so, even if subsequent events cause our views to change. These forward-looking statements should not be relied upon as representing the Company’s views as of any date subsequent to the date of this press release.

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2. NIMH- Prevalence of bipolar disorder in adults. November 2017. Accessed June 24, 2021. https://www.hcp.med.harvard.edu/ncs/ftpdir/NCS-R_12-month_Prevalence_Estimates.pdf
3. Data on File
4. inVibe Patient Agitation Market Research, July 2022 (n=57)
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   Based on Symphony patient level claims analysis. Annual prevalence of diagnosed schizophrenia in the United States was estimated from calculations of an administrative claims database of 3 million privately ensured beneficiaries from 1999 to 2003, California Medicaid claims covering the period 2000-2002, and published statistics in uninsured and veteran populations. Based on diagnostic interview data from National Comorbidity Survey Replication (NCS-R) conducted between February 2001 and April 2003. N=9282 for the main interview, n=5692 for the bipolar disorder subset.