BioXcel Therapeutics Announces Initiation of SERENITY At-Home Pivotal Phase 3 Safety Trial of BXCL501 for Acute Treatment of Agitation Associated with Bipolar Disorders or Schizophrenia

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Estimated 23 million annual agitation episodes in the at-home setting [1-3]

No FDA-approved therapies in the at-home setting for acute treatment of agitation associated with bipolar disorders or schizophrenia

NEW HAVEN, Conn., Sept. 05, 2024 (GLOBE NEWSWIRE) -- BioXcel Therapeutics, Inc. (Nasdaq: BTAI), a biopharmaceutical company utilizing artificial intelligence to develop transformative medicines in neuroscience and immuno-oncology, today announced the initiation of patient enrollment in its SERENITY At-Home pivotal Phase 3 trial. The trial will evaluate the safety of BXCL501, the Company's investigational, proprietary, orally dissolving film formulation of dexmedetomidine, in the at-home setting for the acute treatment of agitation associated with bipolar disorders or schizophrenia. The trial duration currently is expected to be 9 to 12 months.

"The management of agitation for patients at home is extremely important but it is a significant clinical challenge," said Dr. John Krystal, M.D., the Robert L. McNeil, Jr. Professor of Translational Research and Chair of the Department of Psychiatry at Yale School of Medicine. "Treating agitation early at home could help reduce patient suffering and trips to the emergency room, promoting patient safety and reducing the cost of treatment. New therapeutic options could be important in addressing this treatment gap."

"We are pleased to have initiated our SERENITY At-Home trial, which is based on our extensive engagement with the FDA and our considerable previous trial experience with BXCL501. We are also encouraged by the safety results observed for BXCL501 in our programs to date and by the experience of patients and prescribers with IGALMI ™," said Vincent J. O'Neill, M.D., Executive Vice President, Chief of Product Development and Medical Officer of BioXcel Therapeutics. "The primary objective of the study is the characterization of the safety profile in the home setting of the 120 mcg dose, an approved dose of IGALMI."

SERENITY At-Home Pivotal Phase 3 Trial Design Summary
The SERENITY At-Home Phase 3 trial is a double-blind, placebo-controlled study to evaluate the safety of a 120 mcg dose of BXCL501 in the home setting.

- The outpatient trial will enroll approximately 200 patients residing at home either alone or with caregivers/informants with at least one treated episode of agitation.
- Patients will self-administer 120 mcg of BXCL501 or placebo when agitation episodes occur over the 12-week trial period.
- Safety data will be collected from individual subjects as well as caregivers/informants, as is typical for outpatient trials. The investigator or designee will assess and record adverse events at all in-clinic visits based on telephone interviews and agitation episode diaries from patients and (when applicable) caregivers/informants.
- The primary objective is safety. Patients or caregivers/informants will complete a modified clinical global impression of change (mCGI-C) two hours after dosing as an exploratory endpoint to evaluate their impression of use in the outpatient setting.

A corporate presentation, including information on the SERENITY At-Home trial, is available on the Events & Presentations page under the "News/Events" tab in the Investors & Media section of the Company's website at bioxceltherapeutics.com.

About BXCL501
Outside of its approved indication by the U.S. Food and Drug Administration as IGALMI™ (dexmedetomidine) sublingual film, BXCL501 is an investigational proprietary, orally dissolving film formulation of dexmedetomidine, a selective alpha-2 adrenergic receptor agonist. BXCL501 is under investigation by BioXcel Therapeutics for the acute treatment of agitation associated with Alzheimer's dementia and for the acute treatment of agitation associated with bipolar I or II disorder or schizophrenia in the at-home setting. The safety and efficacy of BXCL501 for these investigational uses have not been established. BXCL501 has been granted Breakthrough Therapy designation by the FDA 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 BioXcel Therapeutics, Inc.
BioXcel Therapeutics, Inc. (Nasdaq: BTAI) is a biopharmaceutical company utilizing artificial intelligence to develop transformative medicines in neuroscience. Its wholly owned subsidiary, OnkosXcel Therapeutics, is focused on the development of medicines in 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 indications. 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 and Section 21E of the Securities Exchange Act of 1934, as amended. All statements contained in this press release other than statements of historical fact should be considered forward-looking statements, including, without limitation, statements related to: the Company's advancement of its SERENITY trial and the trial design and expected timing thereof; potential market opportunity for BXCL501; and the potential for the results from the Company's completed, ongoing and proposed clinical trials to support regulatory approvals for its product candidates. 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 operating history; its incurrence of significant losses; its need for substantial additional funding and ability to raise capital when needed; the impact of the reprioritization; its significant indebtedness, ability to comply with covenant obligations and potential payment obligations related to such indebtedness and other contractual obligations; the Company has identified conditions and events that raise substantial doubt about its ability to continue as a going concern; its limited experience in drug discovery and drug development; risks related to the TRANQUILITY program; its dependence on the success and commercialization of IGALMI™, BXCL501, BXCL502, BXCL701 and BXCL702 and other product candidates; the number of episodes of agitation and the size of the Company's total addressable market may be overestimated, and approval that the Company may obtain may be based on a narrower definition of the patient population; its lack of experience in marketing and selling drug products; the risk that IGALMI or the Company's product candidates may not be accepted by physicians or the medical community in general; the Company still faces extensive and ongoing regulatory requirements and obligations for IGALMI; 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; its novel approach to the discovery and development of product candidates based on EvolverAI; the significant influence of and dependence on BioXcel LLC; its exposure to patent infringement lawsuits; its reliance on third parties; its ability to comply with the extensive regulations applicable to it; impacts from data breaches or cyber-attacks, if any; risks associated with the increased scrutiny relating to environmental, social and governance (ESG) matters; risks associated with federal, state or foreign health care "fraud and abuse" laws; and its ability to commercialize its product candidates, as well as the important factors discussed under the caption "Risk Factors" in its Annual Report on Form 10-K for the fiscal year ended December 31, 2023, as such factors may be updated from time to time in its other filings with the SEC, including without limitation its Quarterly Report on Form 10-Q for the quarterly period ended June 30, 2024, 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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References

1. Data on file relating to agitation episodes associated with schizophrenia or bipolar I or II disorder. BioXcel Therapeutics, Inc. New Haven, CT December 2020. Episode estimations may not reflect potential treatable episodes, and actual addressable market may be smaller.
2. Data from Wu EQ, Shi L, Birnbaum H, et al. Annual prevalence of diagnosed schizophrenia in the USA: a claims data analysis approach. Psychol Med. 2006;36(11):1535-1540. Estimates based on whether indications are approved for at-home use for the intended patient population and such patients are treatable. Episode estimations may not reflect potential treatable episodes, and actual addressable market may be smaller.
3. National Institute of Mental Health. Prevalence of bipolar disorder in adults. November 2017. Accessed December 16, 2022. https://www.nimh.nih.gov/health/statistics/bipolar-disorder. Episode estimations may not reflect potential treatable episodes, and actual addressable market may be smaller.