BioXcel Therapeutics Provides Update on its BXCL501 Program for the Acute Treatment of Dementia Related Agitation

March 3, 2021

Review of TRANQUILITY data showed 30 mcg dose met statistical significance across multiple scales

Company initiated supplemental 40 mcg dose cohort to help inform clinical development strategy across the full range of dementia care settings

End of Phase 2 meeting scheduled with U.S. Food and Drug Administration ("FDA") in Q2 2021

Pivotal Phase 3 program expected to begin in the second half of 2021

NEW HAVEN, Conn., March 03, 2021 (GLOBE NEWSWIRE) -- BioXcel Therapeutics, Inc. ("BioXcel" or the "Company") (Nasdaq: BTAI), a clinical-stage biopharmaceutical company utilizing artificial intelligence approaches to develop transformative medicines in neuroscience and immuno-oncology, today announced an update to its BXCL501 dementia program, including clinical updates and the scheduling of its end of Phase 2 meeting with the FDA. BXCL501 is the Company's investigational, proprietary, orally dissolving thin film formulation of dexmedetomidine ("Dex"). Following the end of Phase 2 meeting with the FDA, the Company plans to finalize study design, dosing, and endpoints for its Phase 3 registrational program that is expected to begin in the second half of 2021.

As part of its overall clinical development strategy aimed at addressing the broadest dementia related agitation market, the Company is also providing an update on the TRANQUILITY clinical results, as well as announcing initiation of a supplemental study to evaluate a 40 mcg dose:

- In January 2021, BioXcel announced that the 60 mcg dose of BXCL501 met the primary and all secondary endpoints in the TRANQUILITY Phase 1b/2 study for the acute treatment of dementia related agitation, demonstrating statistically significant, clinically meaningful, rapid, and durable reductions in agitation with no severe or serious adverse events. The Company also reported that a lower 30 mcg dose showed numerical improvements compared to placebo. Following a routine quality control review of the Company's TRANQUILITY study data, the Company discovered that two patients were mis-categorized within the 30 mcg cohort at the clinical site. After moving the two patients into their appropriate placebo and 30 mcg groups, the data from the 30 mcg cohort were re-analyzed, resulting in the 30 mcg dose crossing over to statistical significance at the two hour time point, as measured by PEC: p=0.0149; PAS: p=0.0195; and CMAI: p=0.0364.

- Additionally, BioXcel has initiated a 46 patient, multicenter, placebo-controlled study investigating a 40 mcg dose cohort of BXCL501. PK/PD modeling of BXCL501 data from the TRANQUILITY trial was supportive of evaluating the efficacy of a 40 mcg dose. Results are expected to provide additional insights to support the Company's clinical development strategy directed at all segments of the dementia market, including assisted living facilities and community or home care settings.

"We are extremely pleased with the overall progress made with our BXCL501 program for the acute treatment of dementia related agitation," commented Vimal Mehta, Chief Executive Officer of BioXcel. "We believe BXCL501, with its novel mechanism of action, has the potential to provide a safe and effective therapy for patients and caregivers to use across the full range of dementia care settings. We look forward to discussing our registrational development strategy with the FDA in the second quarter of 2021 and initiating the pivotal BXCL501 dementia program in the second half of 2021."

The updated TRANQUILITY presentation is available on the Investors section of the Company's website at *www.bioxceltherapeutics.com*.

About Dementia Related Agitation

Dementia is a neurocognitive condition caused by damage to brain cells that leads to a decline in cognitive abilities and independent function. It affects approximately 6 million individuals in the United States, with Alzheimer's disease accounting for up to 80% of these cases. During the course of the disease, patients with dementia often suffer from psychological and behavioral symptoms, such as agitation, which has been reported in up to 70% of patients. Agitation associated with dementia can negatively affect both the patient and caregiver's quality of life. Caregiver burden can contribute significantly to burnout, which can result in premature institutionalization of the patient. Treating agitation associated with dementia has been a challenge for providers as there are currently no FDA-approved therapies for the treatment of dementia-related agitation, and off-label therapies have black box warnings associated with their use.

About the Positive and Negative Syndrome Scale-Excitatory Component Score (PEC or PANSS-EC)

The PEC total score is a validated endpoint for measuring acute agitation in schizophrenia and bipolar patients. This scale is used in clinical research to quantify the severity of a patient's acute agitation. The PEC rating evaluates 5 elements associated with agitation: poor impulse control, tension, hostility, uncooperativeness, and excitement; each scored 1 (minimum) to 7 (maximum). The PEC total score is the sum of these 5 elements and thus ranges from 5 to 35.

About the Pittsburgh Agitation Scale (PAS)

PAS is a validated instrument used to monitor the severity of agitation associated with dementia. The PAS measures 4 behavior groups: aberrant vocalization, motor agitation, aggressiveness, and resisting to care. The groups are evaluated on a scale from 0 to 4, with 0 defined as no agitation present and 4 defined as the highest form of agitation. The PAS total score ranges from 0 to 16.

Modified Cohen-Mansfield Agitation Inventory (Mod-CMAI)

The Modified Cohen-Mansfield Agitation (Mod-CMAI) is an inventory consisting of 29 behaviors, each rated on a 7-point scale of frequency with 1 defined as never occurring and 7 defined as several times an hour. Only behaviors manifested by the subject at baseline were assessed throughout the study.

About BXCL501

BXCL501 is an investigational, proprietary, orally dissolving thin film formulation of dexmedetomidine, a selective alpha-2a receptor agonist for the treatment of agitation and opioid withdrawal symptoms. BioXcel believes that BXCL501 directly targets a causal agitation mechanism, and the Company has observed anti-agitation results in multiple clinical studies across several neuropsychiatric disorders. BXCL501 has been granted Fast Track Designation by the U.S. Food and Drug Administration for the acute treatment of agitation in patients with schizophrenia, bipolar disorders, and dementia. BXCL501 has been studied in two Phase 3 trials (SERENITY I and II) for the acute treatment of schizophrenia related agitation and bipolar disorder related agitation, respectively, and in a Phase 1b/2 trial (TRANQUILITY) for the acute treatment of dementia related agitation. This product candidate is also currently being evaluated in a Phase 1b/2 trial (RELEASE) for the treatment of opioid withdrawal symptoms and in a Phase 2 trial (PLACIDITY) for the treatment of delirium related agitation.

BioXcel Therapeutics, Inc.

BioXcel Therapeutics, Inc. is a clinical-stage biopharmaceutical company utilizing artificial intelligence approaches to develop transformative medicines in neuroscience and immuno-oncology. BioXcel'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. BioXcel's two most advanced clinical development programs are BXCL501, an investigational, proprietary, orally dissolving thin film formulation of dexmedetomidine for the treatment of agitation and opioid withdrawal symptoms, and BXCL701, an investigational, orally administered, systemic innate immunity activator in development for the treatment of aggressive forms of prostate cancer and advanced solid tumors that are refractory or treatment naïve to checkpoint inhibitors. For more information, please visit www.bioxceltherapeutics.com.

Forward-Looking Statements

This press release includes "forward-looking statements" within the meaning of the Private Securities Litigation Reform Act of 1995. Forward-looking statements in this press release include but are not limited to the timing and data from clinical development initiatives and trials for BXCL501 for the treatment of dementia related agitation, the Company's planned discussions with the FDA and the future clinical development strategy for BXCL501. When used herein, words including "anticipate," "being," "will," "plan," "may," "continue," and similar expressions are intended to identify forward-looking statements. 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 BioXcel's current expectations and various assumptions. BioXcel believes there is a reasonable basis for its expectations and beliefs, but they are inherently uncertain.

BioXcel 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; its limited experience in drug discovery and drug development; its dependence on the success and commercialization of BXCL501 and BXCL701 and other product candidates; 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 BioXcel's product candidates; its approach to the discovery and development of product candidates based on EvolverAI is novel and unproven; its exposure to patent infringement lawsuits; its ability to comply with the extensive regulations applicable to it; impacts from the COVID-19 pandemic; its ability to commercialize its 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, 2020, as such factors may be updated from time to time in its other filings with the SEC, accessible on the SEC's website at www.sec.gov and the Investors section of our website at www.bioxceltherapeutics.com.

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 BioXcel 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 BioXcel's views as of any date subsequent to the date of this press release.

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