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# BioXcel Therapeutics Highlights Upcoming Major Milestones

*Clinical results expected 1H 2020 from BXCL501 Phase 3 trial for acute treatment of agitation in Schizophrenia and Bipolar patients*

*Data also expected from BXCL501 Phase 1b in 1H 2020 for acute treatment of agitation in Geriatric Dementia/Alzheimer's Disease*

*Data readouts expected from BXCL701 in Phase 1b/2 for tNEPC and Pancreatic Cancer trials in Q4 2019 and 1H 2020, respectively*

*Cash position sufficient to fund data readouts from key clinical trials on expected timeline*

NEW HAVEN, Conn., Oct. 10, 2019 (GLOBE NEWSWIRE) -- BioXcel Therapeutics, Inc. ("BTI" or the "Company") (Nasdaq: BTAI), a clinical-stage biopharmaceutical development company utilizing novel artificial intelligence approaches to identify and advance the next wave of medicines in neuroscience and immuno-oncology, today affirmed its expected timeline regarding upcoming milestones.

"We believe our recent equity offering represents a key step in driving the success of our clinical programs and providing sufficient funding to support key data readouts, including clinical results from our lead neuroscience program, BXCL501, as well as our immuno-oncology program, BXCL701," commented Vimal Mehta, Ph.D., Chief Executive Officer of BTI.

BXCL501, a differentiated, novel agent intended for treatment of agitation in neuropsychiatric diseases, demonstrated statistically significant results in a Phase 1b trial in agitated Schizophrenia patients with clinically meaningful, rapid and durable reduction in agitation. In addition, the results from the Phase 1 trial with IV dexmedetomidine in Alzheimer's Disease patients produced rapid calming effects, as previously announced.

"Given there are no FDA approved therapies and that antipsychotics have black-box warnings for elderly patients with dementia, we believe this is an opportune time to continue to advance the clinical development of BXCL501 in geriatric dementia, which has the potential to be a well-tolerated, noninvasive treatment," continued Mehta. "We are further encouraged by the progress we have made in our BXCL701 program, with the first data readout expected from the treatment emergent neuroendocrine prostate cancer program in the fourth quarter of 2019. In addition, we received U.S. Food and Drug Administration Orphan Drug Designation for BXCL701 for the treatment of Acute Myeloid Leukemia, representing a potential expansion of the program into hematological malignancies."

**About BXCL501:**

BXCL501 is an investigational, proprietary sublingual thin film of dexmedetomidine, a

selective alpha2 A -adrenergic agonist designed for the treatment of acute agitation. BTI believes that BXCL501 directly targets a causal agitation mechanism and the Company has observed anti-agitation effects in multiple clinical studies across multiple neuropsychiatric indications. BXCL501 is currently being developed for agitation associated with schizophrenia and bipolar disorders followed by geriatric dementia/Alzheimer's.

**About BXCL701:**

BXCL701 is an investigational orally-available systemic innate immunity activator with dual mechanisms of action. It has shown single agent activity in melanoma and safety has been evaluated in more than 700 healthy subjects and cancer patients. Designed to stimulate both the innate and acquired immune systems, BXCL701 is designed to inhibit dipeptidyl peptidase (DPP) 8/9 and block immune evasion by targeting Fibroblast Activation Protein (FAP). BXCL701 is currently being developed for treatment of a rare form of prostate cancer and for pancreatic cancer in combination with other immuno-oncology agents.

**About BioXcel Therapeutics, Inc.:**

BioXcel Therapeutics, Inc. is a clinical stage biopharmaceutical company focused on drug development that utilizes novel artificial intelligence approaches to identify and advance the next wave of medicines in neuroscience and immuno-oncology. BTI's drug re-innovation approach leverages existing approved drugs and/or clinically evaluated product candidates together with big data and proprietary machine learning algorithms to identify new therapeutic indices. BTI's two most advanced clinical development programs are BXCL501, an investigational sublingual thin film formulation in development for acute treatment of agitation resulting from neuropsychiatric disorders, and BXCL701, an investigational orally administered systemic innate immunity activator in development for treatment of a rare form of prostate cancer and for treatment of pancreatic cancer in combination with other immuno-oncology agents. 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 of the data readouts for the BXCL501 and BXCL701 trials, the Company's wearable digital device initiative and the efficacy of BXCL501 in the treatment of agitation. 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 BTI's current expectations and various assumptions. BTI believes there is a reasonable basis for its expectations and beliefs, but they are inherently uncertain.

BTI 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; 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; 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 June 30, 2019 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 BTI 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 BTI’s views as of any date subsequent to the date of this press release.

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