BioXcel Therapeutics Reports Third Quarter 2021 Financial Results and Recent Operational Highlights

November 10, 2021

Plans for commercial and launch readiness advancing for BXCL501 orally dissolving film, for the acute treatment of agitation associated with schizophrenia and bipolar disorders I and II, ahead of January 5, 2022 PDUFA date

On track to initiate Phase 3 program for acute treatment of agitation in patients with Alzheimer's disease in Q4 2021

Strengthened neuroscience franchise with indication expansion for BXCL501 in major depressive disorder (MDD)

Introduced BXCL502 candidate for chronic agitation in dementia, identified through Company's artificial intelligence platform

Demonstrated encouraging anti-tumor activity and favorable safety profile for BXCL701 in combination with KEYTRUDA® in mCRPC patients with adenocarcinoma; expansion of Phase 2 trial in SCNC cohort

To host conference call today, Nov. 10, 2021, at 8:30 a.m. EDT

NEW HAVEN, Conn., Nov. 10, 2021 (GLOBE NEWSWIRE) -- BioXcel Therapeutics, Inc. (Nasdaq: BTAI), a clinical-stage biopharmaceutical company utilizing artificial intelligence approaches to develop transformative medicines in neuroscience and immuno-oncology, today announced its financial results for the third quarter ended September 30, 2021 and provided an update on key strategic initiatives.

"We have made tremendous progress advancing our neuroscience and immuno-oncology franchises," said Vimal Mehta, Ph.D., CEO of BioXcel Therapeutics. "With our PDUFA date for BXCL501 less than two months away, we are excited that our commercial and launch readiness planning is progressing well. We are equally excited about initiating our Phase 3 program for BXCL501 for acute treatment of agitation associated with dementia in Alzheimer's patients, the most prevalent type of dementia in the United States, which has a high unmet medical need and we believe would mark the first orally available FDA-approved therapy. Furthermore, adding major depressive disorder as a potential indication for BXCL501 and introducing BXCL502 as a candidate for chronic treatment of agitation in dementia strengthens our pipeline and expands our market opportunity."

Dr. Mehta added, "Our immuno-oncology program is also advancing, with positive interim results from our Phase 2 trial of BXCL701 in heavily pre-treated metastatic castration-resistant prostate cancer (mCRPC) demonstrating encouraging efficacy and a favorable safety profile. We believe BXCL701 shows considerable potential as an investigational, orally administered, systemic innate immune activator."

Company Highlights

Neuroscience Franchise

BXCL501 is an investigational, proprietary, orally dissolving, thin film formulation of the adrenergic receptor agonist dexmedetomidine for the treatment of agitation associated with neuropsychiatric disorders. BXCL501 has received FDA Breakthrough Therapy designation for the acute treatment of agitation associated with dementia and FDA Fast Track designation for the acute treatment of agitation associated with schizophrenia, bipolar disorders I and II, and dementia.

- **BXCL501 for Acute Treatment of Agitation Associated with Schizophrenia and Bipolar Disorders I and II:** On track with FDA review of BXCL501 NDA for acute treatment of agitation associated with schizophrenia and bipolar disorders I and II; Marketing Authorization Application to European Medicines Agency of BXCL501 expected to be submitted in 1H 2022.

- **BXCL501 for Acute Treatment of Agitation in Patients with Alzheimer's Disease:** Following multiple meetings with FDA and alignment on key design features, on track to initiate Phase 3 program in Q4 2021. Alzheimer's disease is the most prevalent type of dementia in the U.S. and is expected to double from 5.8 million patients in 2020 to 11.8 million patients by 2040.[1] The Company remains interested in exploring BXCL501 for other dementia subtypes as part of future development.

- **BXCL501 for Major Depressive Disorder (MDD):** Held pre-Investigational New Drug (IND) meeting with FDA for use of BXCL501 as an adjunctive treatment for MDD, with Selective Serotonin Reuptake Inhibitors (SSRIs) and serotonin-norepinephrine reuptake Inhibitors (SNRIs), and to align on key design features; preparing to submit IND and expect to initiate a clinical trial in 1H 2022.

- **BXCL502 for Chronic Treatment of Agitation in Patients with Dementia**: Formulation and clinical development planning underway for BXCL502 as a potential monotherapy and in combination with BXCL501 for chronic treatment of agitation in patients with dementia; designed to be a potent and selective antagonist for a GPCR target affecting serotonergic signaling

in the cerebral cortex.

AI-driven Drug Discovery & Development

- Hosted a Virtual R&D Day in September highlighting the Company's innovative approaches to leveraging its proprietary artificial intelligence platform to expand current neuroscience portfolio, including identification of the Company's newest product candidate, BXCL502, and to broaden the addressable market for lead program, BXCL501, in MDD.

1. Alzheimer's Association.

Immuno-Oncology Franchise

BXCL701 is an investigational, orally administered, systemic innate immune 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.

- **Metastatic Castration-Resistant Prostate Cancer (mCRPC) Program:** Presented positive interim data from Phase 1b/2 trial of BXCL701 in combination with KEYTRUDA® (pembrolizumab) for heavily pre-treated mCRPC patients with adenocarcinoma at the 2021 European Society for Medical Oncology Congress in September. Following this data, announced expansion of ongoing Phase 1b/2 trial of BXCL701 in mCRPC patients with either de novo or treatment-emergent small-cell neuroendocrine carcinoma (SCNC).
- **Solid Tumors Program (Checkpoint Naïve and Refractory):** Expect to present additional efficacy data from MD Anderson-led open-label Phase 2 basket trial of BXCL701 and KEYTRUDA® in 1H 2022.
- **Peer-Reviewed Journal Findings Published on BXCL701 Mechanism of Action:** *Journal of Immunotherapy of Cancer* reported data findings, on November 4, 2021, suggesting BXCL701 may enhance immunotherapy efficacy in 'cold' tumor types such as pancreatic cancer. These findings also highlight the potential importance of natural killer (NK) cells along with T cells in regulating pancreatic cancer tumor growth.

Commercial and Launch Readiness Progress

- **Expanded Sales Leadership:** onboarded a Vice President of Sales and Regional Sales Directors; continuing to recruit sales force across key territories.
- **Optimizing Market Access and Pricing Strategy for BXCL501**: through evidence-based market research.
- **Fully Launched Unbranded Disease Education Campaign (Including partnersincalm website):** to promote awareness of acute agitation in schizophrenia and bipolar disorders.

Medical Affairs Progress

- **Medical Science Liaison and Medical Managed Care Teams Fully Deployed:** actively engaged with healthcare professionals and payers to provide key insights and support potential BXCL501 commercial launch, including participation and presentations at:
  - Neuroscience Education Institute Congress in November
  - Psych Congress, American College of Emergency Physicians conference and Academy of Managed Care Pharmacy conference in October
  - Emergency Nurses Association Annual Meeting in September

Third Quarter 2021 Financial Results

Research and Development Expenses: Research and development expenses were $11.9 million during the third quarter of 2021, as compared to $16.3 million for the same period in 2020. The decreased expenses were primarily attributable to a reduction in BXCL501 clinical trial costs offset in part by increased BXCL701 trial costs. In addition, the Company experienced greater professional and consulting fees primarily related to BXCL501 development.

General and Administrative Expenses: General and administrative expenses were $14.9 million for the third quarter of 2021, as compared to $8.5 million for the same period in 2020. The increase was primarily due to higher stock-based compensation and personnel costs due to continued expansion of teams, increased marketing and commercial costs related to the potential launch of BXCL501 in the U.S., as well as increased legal and professional fees, and insurance costs.

Net Loss: BioXcel Therapeutics reported a net loss of $26.8 million for the third quarter of 2021, compared to a net loss of $24.8 million for the same period in 2020.

As of September 30, 2021, cash and cash equivalents totaled approximately $252.9 million.

Conference Call

BioXcel Therapeutics will host a conference call and webcast today at 8:30 a.m. EDT to discuss its third quarter 2021 financial results and provide an update on recent operational highlights. To access the call, please dial 877-407-5795 (domestic) and 201-689-8722 (international). A live webcast of the call will be available on the Investors section of the BioXcel website, www.bioxceltherapeutics.com, and a replay of the call will be available through at least December 11, 2021.

BioXcel Therapeutics may use its website as a distribution channel of material information about the Company. Financial and other important information regarding the Company is routinely posted on and accessible through the Investors sections of its website at www.bioxceltherapeutics.com. In addition, you may automatically receive email alerts and other information about the Company when you enroll your email address by visiting the “Email Alerts” option under the News/Events menu of the Investors & Media section of its website.

About 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 Therapeutics’ two most advanced clinical development programs are BXCL501, an investigational, proprietary, orally dissolving thin film formulation of dexmedetomidine for the treatment of agitation associated with psychiatric and neurological disorders, 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 trials for BXCL501 and BXCL701, the timing of potential commercial approval of BXCL501 for the acute treatment of schizophrenia and bipolar disorders I and II, the Company’s planned commercial structure and medical affairs strategy, the timing of the Company’s MAA application for BXCL501, the potential value of BXCL501, BXCL502 and BXCL701 as treatment options, and future financial and operational results. When used herein, words including “anticipate,” “will,” “plan,” “may,” “continue,” “intend,” “designed,” “goal” 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 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; its limited experience in drug discovery and drug development; its dependence on the success and commercialization of BXCL501, BXCL502 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 the Company’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, 2021, 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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BioXcel Therapeutics, Inc (BTAI)

Statements of Operations
(Unaudited, in thousands, except per share amounts)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2021	2020	2021	2020
Revenues	$ -	$ -	$ -	$ -
Operating expenses:				
Research and development	11,933	16,317	40,183	46,595
General and administrative	14,879	8,451	40,621	14,605
Total operating expenses	26,812	24,768	80,804	61,200
Loss from Operations	(26,812)	(24,768)	(80,804)	(61,200)
Other income (expense)				
Interest income	12	20	32	140
Interest expense	(11)	(5)	(34)	(23)
Net loss	$ (26,811)	$ (24,753)	$ (80,806)	$ (61,083)
Net loss per share - basic and diluted	$ (0.96)	$ (1.07)	$ (3.13)	$ (2.94)
Weighted average shares outstanding - basic and diluted	27,972	23,050	25,832	20,779

Condensed Balance Sheets
(Unaudited, in thousands)

	September 30, 2021	December 31, 2020
Cash and cash equivalents	252,912	213,119
Working Capital	242,333	205,223
Total assets	261,332	219,936
Long-term liabilities	1,180	1,398
Total liabilities	15,776	13,240
Total stockholders' equity	245,556	206,696