BioXcel Therapeutics Reports Fourth Quarter and Full Year 2021 Financial Results and Recent Operational Highlights

March 10, 2022

Ready to launch BXCL501 upon approval as Company nears April 5, 2022 PDUFA action date

Initiated TRANQUILITY II AND III pivotal Phase 3 trials for BXCL501 in acute treatment of agitation associated with Alzheimer's disease

Submitted IND to evaluate BXCL501 as adjunctive treatment for major depressive disorder (MDD)

Demonstrated encouraging composite response rates for BXCL701 in combination with KEYTRUDA® for mCRPC in SCNC and adenocarcinoma cohorts

To host conference call today, March 10, 2022, at 8:30 a.m. ET

NEW HAVEN, Conn., March 10, 2022 (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 fourth quarter and full year ended December 31, 2021 and provided an update on key strategic initiatives.

"2021 was marked by significant achievement across the business, most notably with the NDA submission for BXCL501, which concluded an unprecedented, less than three-year journey from first-in-human trials to regulatory submission," said Vimal Mehta, Ph.D., CEO of BioXcel Therapeutics. "As we near potential approval, we remain focused on executing our launch readiness plan. Matt Wiley, who recently joined as our Chief Commercial Officer, has, and will continue to play a pivotal role in developing our commercial and market access strategy and building out our field sales organization. In parallel, we continued to advance our 'land and expand' strategy by furthering our Alzheimer's disease and MDD programs, both important components of our five-year vision to become the leading AI-enabled neuroscience Company."

Dr. Mehta added, "Our oncology franchise further progressed with encouraging composite response rates for BXCL701 in combination with KEYTRUDA® for mCRPC patients with SCNC and adenocarcinoma phenotypes, both aggressive tumors with very few treatment options. We continue to develop BXCL701's potential to convert cold tumors to hot as a novel 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 U.S. Food & Drug Administration (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 the Acute Treatment of Agitation in Patients with Schizophrenia and Bipolar Disorders:** The Company is gearing up to launch BXCL501 in the U.S. pending FDA approval by the April 5, 2022 PDUFA action date. This is a large addressable market, with over 7 million people in the U.S. diagnosed with schizophrenia or bipolar disorders[1-3], totaling approximately 25 million annual agitation episodes.[3]

- **Data Published in Journal of the American Medical Association (JAMA) from SERENITY II Pivotal Phase 3 Trial Evaluating BXCL501 in Bipolar Disorders:** Results demonstrate BXCL501 as a potential treatment for the millions of patients experiencing agitation, a difficult-to-manage symptom associated with many neuropsychiatric conditions.

Indication Expansion

- **BXCL501 for Acute Treatment of Agitation in Patients with Alzheimer's Disease:** Initiated pivotal Phase 3 program in Q4 2021 following alignment with FDA on key trial design features. Alzheimer's disease is the most prevalent type of dementia in the U.S. and is expected to approximately double from 5.8 million patients in 2020 to 11.8 million patients by 2040.[4]

- **BXCL501 for Major Depressive Disorder (MDD):** Submitted an Investigational New Drug (IND) application to evaluate BXCL501 as an adjunct treatment for MDD, the most common type of depression in the United States with approximately 27 million cases a year.[5] There are over 300 million antidepressant prescriptions filled annually, with limitations of current treatment including slow onset of action and incomplete response.[6]

Geographic Expansion

- **International Growth:** The Company expects to submit a Marketing Authorization Application to the European Medicines Agency (EMA) for BXCL501 in 1H 2022.

AI-driven Drug Discovery & Development

- The Company continues its innovative approach to R&D by leveraging its proprietary artificial intelligence platform to expand its current neuroscience portfolio, including identification of the Company's newest product candidate, BXCL502. The Company initiated formulation for this new product candidate for chronic treatment of agitation in dementia patients. BXCL502 is designed to be a potent and selective antagonist for a GPCR target affecting serotonergic signaling in the cerebral cortex.

BXCL501 Commercial Readiness Progress

- **Expanded Commercial Organization:** Hired Chief Commercial Officer Matt Wiley to develop integrated commercial strategy for potential BXCL501 approval and beyond, and continue the buildout of the Company's national sales force.

- **Medical Science Liaison and Medical Managed Care Teams Fully Deployed:** Continued engagement with health care professionals (HCPs) and payers to provide key insights and support potential BXCL501 commercial launch, including planned participation at:
  - Academy of Managed Care Pharmacy annual meeting in March 2022
  - International Society of Pharmacoeconomics and Outcomes Research conference in May 2022

Immuno-Oncology Franchise

BXCL701 is an investigational, orally administered, systemic innate immune activator in development for 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 data from Phase 2 trial of BXCL701 in aggressive forms of prostate cancer at 2022 ASCO Genitourinary Cancers Symposium in February. The presentations showed that BXCL701 plus KEYTRUDA® (pembrolizumab) demonstrated encouraging composite response rates in patients with either adenocarcinoma (21% response rate) or SCNC (33% response rate) phenotype, supporting further evaluation of BXCL701's potential to extend checkpoint inhibitor therapy into cold tumor settings.

- **Solid Tumors Program (Checkpoint Naïve and Refractory):** Additional efficacy data from MD Anderson-led open-label Phase 2 basket trial of BXCL701 and KEYTRUDA® expected in 2H 2022.

Fourth Quarter and Full Year 2021 Financial Results

Research and Development Expenses: Research and development expenses were $12.5 million for the fourth quarter of 2021, compared to $11.4 million for the same period in 2020. The increased expenses were primarily attributable to increased headcount and related costs during the three-month period of 2021.

Research and development expenses were $52.7 million for the full year 2021, as compared to $58.0 million for the same period in 2020. The decrease for the year ended December 31, 2021 was primarily attributable to decreased clinical trial expenses related to BXCL501, partially offset by increased costs related to BXCL701 clinical trials.

General and Administrative Expenses: General and administrative expenses were $13.6 million for the fourth quarter of 2021, as compared to $9.7 million for the same period in 2020. The increase was primarily due to increased headcount and related costs, including higher stock-based compensation, 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.

General and administrative expenses were $54.2 million for the full year 2021, as compared to $24.3 million for the same period in 2020. The increase for the year ended December 31, 2021 was primarily attributable to increased headcount and associated costs, including non-cash stock-based compensation. The Company also incurred significant commercial costs in preparation for a potential U.S. launch of BXCL501.

Net Loss: BioXcel Therapeutics reported a net loss of $26.1 million for the fourth quarter of 2021, compared to a net loss of $21.1 million for the same period in 2020. For the full year, BioXcel reported a net loss of $106.9 million, compared to a net loss of $82.2 million for the same period in 2020.

As of December 31, 2021, cash and cash equivalents totaled approximately $233.0 million.

Conference Call

BioXcel Therapeutics will host a conference call and webcast March 10, 2022 at 8:30 a.m. ET, to discuss its fourth quarter and full year 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 April 10, 2022.

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. 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. 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, upcoming presentations 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 and the fact that it has never had a drug approved; 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 December 31, 2021, as such factors may be updated from time to time in its other filings with the SEC, including its Annual Report in Form 10-K for the fiscal year ended December 31, 2021, 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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1. 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.
2. UN Population Prospectus. Retrieved May 6, 2021. https://population.un.org/wpp.
3. Prevalence of bipolar disorder in adults. November 2017. https://www.hcp.med.harvard.edu/ncs/ftpdir/NCS-R_12-month_Prevalence_Estimates.pdf. Accessed June 24, 2021.
4. Alzheimer’s Association.
5. GlobalData. Major Depressive Disorder: Market Analysis and Forecast to 2029, March 2021.
6. NIH/WHO, SAMHSA, NIMH

BioXcel Therapeutics, Inc.

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

	Three Months Ended December 31,		Year Ended December 31,	
	2021	2020	2021	2020
Revenues	$ -	$ -	$ -	$ -
Operating expenses:				
Research and Development	12,525	11,401	52,708	57,995
General and administrative	13,606	9,696	54,227	24,302
Total operating expenses	26,131	21,097	106,935	82,297
Loss from Operations	(26,131)	(21,097)	(106,935)	(82,297)
Other income (expense)				
Interest income, net	6	11	4	128
Net loss	(26,125)	$ (21,086)	(106,931)	$ (82,169)
Net loss per share- basic and diluted	(0.93)	$ (0.87)	$ (4.05)	$ (3.79)
Weighted average shares outstanding - basic and diluted	27,980	24,375	26,373	21,683

Condensed Balance Sheets
(Unaudited, in thousands)

	December 31, 2021	December 31, 2020
Cash and cash equivalents	232,968	213,119
Working Capital	220,145	205,223
Total assets	239,439	219,936
Long-term liabilities	1,105	1,398
Total liabilities	17,772	13,240
Total stockholders' equity	221,667	206,696