May 10, 2021

BioXcel Therapeutics Reports First Quarter 2021 Financial Results and Provides Business Update

Granted FDA Breakthrough Therapy designation for BXCL501 for the acute treatment of agitation associated with dementia; registrational program expected to begin in 2H 2021

Submitted NDA to FDA for BXCL501 for the acute treatment of agitation associated with schizophrenia and bipolar disorders

Strategic geographic market extension plans for BXCL501 underway; expects to submit MAA to EMA for the acute treatment of agitation associated with schizophrenia and bipolar disorders in 2H 2021

Commercial readiness preparations ramping-up for potential approval of BXCL501 in first two indications

Company to host conference call today at 8:30 a.m. ET

NEW HAVEN, Conn., May 10, 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 its quarterly results for the first quarter ended March 31, 2021 and provided an update on key strategic and operational initiatives.

"This quarter, we have remained committed to executing on our vision for our neuroscience franchise, as we continue to advance the development of BXCL501 for treatment of agitation associated with dementia and prepare for its potential approval in acute agitation associated with schizophrenia and bipolar disorders," stated Vimal Mehta, Chief Executive Officer of BioXcel. "We are pleased to have recently received Breakthrough Therapy designation for BXCL501 in agitation associated with dementia, highlighting the urgent need for new therapy options, as well as this candidate's potential to provide a solution across treatment settings. While we solidify our plans for a pivotal program in this condition, we are committed to building the commercial infrastructure needed to successfully launch BXCL501 in the first two indications, in addition to follow-on indications in the future."

Dr. Mehta continued, "Importantly, we are moving forward with our strategic geographic expansion strategy, with plans to file a Marketing Authorization Application to the European Medicines Agency for BXCL501 for the acute treatment of agitation associated with schizophrenia and bipolar disorders in the second half of 2021. As we continue to advance our innovative neuroscience program, we remain committed to delivering transformative medicines to patients, expanding our regulatory footprint and creating value for our stockholders."

First Quarter 2021 and Recent Highlights

BXCL501-Neuroscience Program

BXCL501 is an investigational, proprietary, orally dissolving thin film formulation of dexmedetomidine, a selective alpha-2 adrenergic receptor agonist, designed for the treatment of agitation and opioid withdrawal symptoms. The Company believes BXCL501 may directly target a causal agitation mechanism.

- Reported positive topline results from the Phase 1b/2 TRANQUILITY trial of BXCL501 for the acute treatment of agitation associated with dementia.
- Granted Breakthrough Therapy designation by the U.S. Food and Drug Administration ("FDA") to BXCL501 for the acute treatment of agitation associated with dementia.
- End of Phase 2 meeting with the FDA planned in 2Q 2021 to discuss registrational trial.
- Recruitment in 40 mcg supplemental dose cohort of BXCL501 in TRANQUILITY expansion study is progressing well.
- Completed its New Drug Application ("NDA") submission to the FDA for BXCL501 for the acute treatment of agitation associated with schizophrenia and the acute treatment of agitation associated with bipolar disorders I and II.
- Plan to submit Marketing Authorization Application ("MAA") with European Medicines Agency ("EMA") using SERENITY I and II data for the acute treatment of agitation associated with schizophrenia and bipolar disorders I and II in 2H 2021.
- Reported results from the Phase 1b/2 study of BXCL501 for the treatment of opioid withdrawal symptoms. Primary safety endpoints were met, and numerically improved retention rates, a secondary endpoint, were demonstrated in multiple BXCL501 dose cohorts.
- PLACIDITY enrollment was voluntarily paused to assess challenges posed in opening relevant clinical sites and enrolling delirium patients in the ICU setting, including as a result of the burden COVID-19 has placed on ICUs.
- Strengthened BXCL501 IP portfolio with issuance of two Japanese patents – Patent No. 6868698, directed to methods of treating agitation, which will expire no earlier than 2037, and Patent No. 1681960, directed to film design, which will expire no earlier than 2041.

Commercial Highlights

- Launched "Boiling Point" educational campaign to raise awareness about agitation associated with schizophrenia and bipolar disorder and its impact on patients and clinicians.
- Completed U.S. market assessment initiatives and design of sales force size and structure.

Medical Affairs Initiatives

- Deployed the Medical Science Liaison and Medical Manage Care teams and have begun scientific and medical-to-medical exchange with healthcare professionals and payers, respectively.
- Presented two posters covering data from the pivotal Phase 3 SERENITY trials in patients with acute agitation associated with schizophrenia and bipolar disorders I and

II at the American Psychiatric Association (“APA”) Annual Meeting.

BXCL701-Immuno-Oncology Program

BXCL701 is an orally-delivered small molecule, immunomodulator designed to inhibit dipeptidyl peptidase ("DPP") 8/9 and block immune evasion by targeting Fibroblast Activation Protein ("FAP"). It has shown single agent anti-tumor activity in melanoma and safety has been evaluated in more than 700 healthy subjects and cancer patients.

- The adenocarcinoma cohort of the ongoing Phase 1b/2 trial of BXCL701 and pembrolizumab (KEYTRUDA®) in aggressive forms of prostate cancer has met its efficacy bar to move to stage two. The trial will now continue to full enrollment and more complete efficacy data are expected to be presented at a scientific conference later this year.
- Granted orphan drug designation by the FDA to BXCL701 for the treatment of soft tissue sarcoma.

Corporate Highlights

- Appointed June Bray to the Company’s Board of Directors. Ms. Bray brings over forty years of extensive U.S. and global regulatory experience across all therapeutics areas, including psychiatry and neurology.
- Appointed Javier Rodriguez as Chief Legal Officer and Corporate Secretary. Mr. Rodriguez brings over 20 years of extensive strategic and legal experience within the biopharmaceutical industry.

First Quarter 2021 Financial Results

Research and Development Expenses: Research and development expenses were $14.7 million during the first quarter of 2021, as compared to $12.4 million for the same period in 2020. The higher expenses were primarily attributable to an increase in personnel and related costs necessary to enlarge our development and medical teams. In addition, we experienced increased professional fees in conjunction with higher consulting fees and CMC costs related to BXCL501, as well as increased costs related to our RELEASE clinical trial. These increases were offset in part by a decrease in SERENITY I and II clinical trial costs.

General and Administrative Expenses: General and administrative expenses were $11.6 million for the first quarter of 2021, as compared to $2.6 million for the same period in 2020. The increase was primarily due to higher personnel related costs as well as costs related to our expansion in preparation of the potential commercial launch of BXCL501 in the U.S., and increased legal, professional fees, and insurance costs.

Net Loss: BioXcel reported a net loss of $26.4 million for the first quarter of 2021, compared to a net loss of $14.9 million for the same period in 2020.

The first quarter 2021 results include approximately $5.6 million in non-cash stock-based compensation costs, compared to non-cash stock-based compensation of $776,000 for the same period in 2020.

As of March 31, 2021, cash and cash equivalents totaled approximately $194 million.

Conference Call

BioXcel will host a conference call and webcast today at 8:30 a.m. ET. To access the call, please dial 877-407-2985 (domestic) and 201-378-4915 (international). A live webcast of the call will be available on the Investors sections of the BioXcel website and a replay of the call will be available through at least May 24, 2021. BioXcel Therapeutics website is available at www.bioxceltherapeutics.com.

BioXcel 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 section of its website at www.bioxceltherapeutics.com.

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'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 trials for BXCL501 and BXCL701, the Company's planned commercial structure, the potential value of BXCL501 and BXCL701 as treatment options, the Company's geographic expansion program for BXCL501, delivering transformative medicines to patients, expanding the Company's regulatory footprint and creating value for stockholders. 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 Quarterly Report on Form 10-Q for the quarterly period ended March 31, 2021, as such factors may further 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 BioXcel's 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.

BioXcel Therapeutics, Inc. (BTAI)

Statement of Operations

(Unaudited, in thousands, except per share amounts)

	Three Months Ended Month 31,	
	2021	**2020**
Revenues	$ –	$ –
Operating Expenses		
Research and Development	14,741	12,371
General and administrative	11,638	2,625
Total operating expenses	26,379	14,996
Loss from Operations	(26,379)	(14,996)
Other Income (expense)		
Interest income	10	91
Interest expense	(7)	(6)

Net loss	$ (26,376)	$ (14,911)
Net loss per – basic and diluted	$ (1.08)	$ (0.79)
Weighted average shares outstanding – basic and diluted	24,524	18,968

BioXcel Therapeutics, Inc.

Condensed Balance Sheet

(Unaudited, in thousands)

	March 31, 2021	December 31, 2020
Cash and cash equivalents	194,015	213,119
Working capital	184,459	205,223
Total assets	200,389	219,936
Long-term liabilities	1,328	1,398
Total liabilities	13,652	13,240
Total stockholders' equity	186,737	206,696

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Source: BioXcel Therapeutics