March 9, 2020

BioXcel Therapeutics Reports Fourth Quarter and Full Year 2019 Financial Results and Provides Business Update

Key BXCL501 data readouts expected in mid-2020 – including results from the SERENITY program and the Phase 1b/2 TRANQUILITY trial in geriatric dementia

Strengthened balance sheet through follow-on offering raising $60 million in net proceeds

NEW HAVEN, Conn., March 09, 2020 (GLOBE NEWSWIRE) -- BioXcel Therapeutics, Inc. ("BTI" or "Company") (Nasdaq: BTAI), a clinical-stage biopharmaceutical company utilizing artificial intelligence to identify improved therapies in neuroscience and immuno-oncology, today announced its quarterly results for the fourth quarter and full year ended December 31, 2019 and provided an update on key strategic and operational initiatives.

"2019 has been a tremendous year for BTI. We have made significant growth in our two programs – BXCL501 and BXCL701 – laying the groundwork to achieve key milestones in the coming years," stated Vimal Mehta, Chief Executive Officer of BTAI. "In neuroscience, we have made momentous advancements in the clinical development of BXCL501 and look forward to announcing topline results from our SERENITY program and our Phase 1b/2 TRANQUILITY trial in dementia-related agitation in mid-2020. Additionally, we have been dedicated to expanding the potential therapeutic use of BXCL501, announcing a fourth indication last month as well as examining biomarkers that may have relevance for a range of hyperarousal disorders. We believe these therapeutic opportunities, along with our plans to investigate BXCL501 for the treatment of all types of agitation, are crucial steps to building out a leading neuroscience franchise."

Dr. Mehta added, "In addition to our ongoing studies with BXCL701 in prostate and pancreatic cancers, we are also evaluating this immuno-oncology candidate, in combination with KEYTRUDA®, in multiple advanced solid tumors with the goal of improving treatment responses to this PD-1 inhibitor. We believe this basket trial, led by researchers at MD Anderson, will help to accelerate the evaluation of BXCL701 and help to explore its full potential."

Fourth Quarter 2019 and Recent Highlights

BXCL501-Neuroscience Program
BXCL501 is an investigational sublingual thin film of dexmedetomidine, a selective alpha-2A adrenergic receptor agonist, designed for the treatment of acute agitation. The Company believes BXCL501 may directly target a causal agitation mechanism.

- We initiated pivotal Phase 3 trials for the acute treatment of agitation in patients with schizophrenia (SERENITY I) and bipolar disorder (SERENITY II). Enrollment of

patients is on track and topline data readouts from both trials are expected mid-2020;
- In January, the first patient was enrolled in the TRANQUILITY study, a Phase 1b/2 trial of BXCL501 for the acute treatment of agitation associated with geriatric dementia, expanding potential therapeutic use of BXCL501 beyond current neuropsychiatric disorders. BTI expects to report data in mid-2020;
- We received clearance from the U.S. Food and Drug Administration for an Investigational New Drug application for the treatment of opioid withdrawal symptoms, a potential fourth indication for BXCL501;
- A Phase 2 study designed to measure biomarkers associated with agitation in patients with schizophrenia and their response to treatment with BXCL501 was initiated by researchers at Yale University earlier last month, with data expected in Q2 2020.

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

- We advanced the clinical evaluation of BXCL701 via the initiation of an open-label Phase 2 basket trial, which is being conducted at MD Anderson. This study is evaluating the combination of BXCL701 and Pembrolizumab (KEYTRUDA®) in patients with advanced solid cancers;
- The Company recently presented additional safety and tolerability data from the first and second patient cohorts of the Phase 1b/2 trial of BXCL701 and KEYTRUDA® for tNEPC at the American Society of Clinical Oncology Genitourinary Cancers Symposium (ASCO GU). The trial is currently enrolling an expansion cohort to explore the use of BID dosing. After the successful optimization of BID dosing, the Company expects to advance to the efficacy stage of the trial;
- The BXCL701 phase of the triple combination study of BXCL701, bempegaldesleukin (NKTR-214, Nektar Therapeutics, Inc.) and BAVENCIO® (avelumab, Merck KGaA, Darmstadt, Germany and Pfizer) in pancreatic cancer is expected to be initiated following Nektar and Pfizer's safety run-in trial of a double combination of bempegaldesleukin and avelumab and the outcome of that trial.

Strengthened Balance Sheet

- In February 2020, the Company raised net proceeds of $60 million in connection with its common stock offering. We believe that these proceeds, together with current reserves, provide BTI cash runway to fund key clinical, regulatory and operational milestones into 2021.

Fourth Quarter and Full Year 2019 Financial Results

BTI reported a net loss of $8.3 million for the fourth quarter of 2019, compared to a net loss of $7.1 million for the same period in 2018. The fourth quarter 2019 results include approximately $0.7 million in non-cash stock based compensation.

Research and development expenses were $6.5 million for the fourth quarter of 2019, as compared to $6.0 million for the same period in 2018. The increase was primarily due to an

increase in professional research and related project costs, salary, and related payroll costs, manufacturing costs offset in part by a decrease in clinical trial expenses.

General and administrative expenses were $1.9 million for the fourth quarter of 2019, as compared to $1.3 million for the same period in 2018. The increase was primarily due to increases in salary, and related payroll costs and professional fees.

BTI reported a net loss of $33.0 million for the full year 2019, compared to a net loss of $19.3 million for the same period in 2018.

Research and development expenses were $25.8 million for full year 2019, as compared to $14.6 million for the same period in 2018. The increase was primarily due to clinical trial costs, salary and related payroll costs, professional research and project costs and manufacturing costs.

General and administrative expenses were $7.8 million for full year 2019, as compared to $5.4 million for the same period in 2018. The increase was primarily due to salary and related payroll costs and professional fees.

As of December 31, 2019, cash and cash equivalents totaled approximately $32.4 million.

Please note that these numbers do not include our recent financing, which secured $60 million in net proceeds.

Conference Call:
BTI 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 BTI website at www.bioxceltherapeutics.com. The replay will be available through March 23, 2020.

About BioXcel Therapeutics, Inc.:

BioXcel Therapeutics, Inc. is a clinical stage biopharmaceutical company focused on drug development that utilizes artificial intelligence to identify improved therapies 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 and data from clinical development initiatives and trials for BXCL501 and BXCL701, the Company’s cash runway and the Company’s future growth and position to execute on key milestones. 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; undesirable side effects caused by BTI’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; its ability to commercialize its product candidates; and the other important factors discussed under the caption “Risk Factors” in its Annual Report on Form 10-K for the fiscal year ended December 31, 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.

BIOXCEL THERAPEUTICS, INC.

BALANCE SHEETS

(amounts in thousands, except share and per share data)

	December 31, 2019	December 31, 2018
ASSETS		
Current assets		
Cash and cash equivalents	$ 32,426	$ 42,565
Prepaid expenses and other current assets	1,681	491

Due from Parent	—	115
Total current assets	34,107	43,171
Property and equipment, net	1,041	327
Operating lease right-of-use asset	1,193	—
Other assets	51	51
Total assets	**$ 36,392**	**$ 43,549**
LIABILITIES AND STOCKHOLDERS' EQUITY		
Current liabilities		
Accounts payable	$ 4,953	$ 1,604
Accrued expenses	3,120	3,056
Due to Parent	64	—
Other current liabilities	331	—
Total current liabilities	8,468	4,660
Operating lease liability	1,029	—
Total liabilities	**9,497**	**4,660**
Stockholders' equity		
Preferred stock, $0.001 par value, 10,000,000 shares authorized; no shares issued or outstanding	—	—
Common stock, $0.001 par value, 50,000,000 shares authorized; 18,087,382 and 15,663,221 shares issued and outstanding as of December 31, 2019 and December 31, 2018, respectively	18	16
Additional paid-in-capital	83,565	62,593
Accumulated deficit	(56,688)	(23,720)
Total stockholders' equity	26,895	38,889
Total liabilities and stockholders' equity	**$ 36,392**	**$ 43,549**

BIOXCEL THERAPEUTICS, INC.

STATEMENTS OF OPERATIONS

(amounts in thousands, except share and per share data)

	2019	2018
Revenues	$ —	$ —
Operating costs and expenses		
Research and development	25,797	14,558
General and administrative	7,804	5,404
Total operating expenses	33,601	19,962
Loss from operations	(33,601)	(19,962)
Other income		
Dividend and interest income, net	633	692
Net loss	$ (32,968)	$ (19,270)

Net loss per share attributable to common stockholders/ Parent basic and diluted	$ (2.02)	$ (1.32)
Weighted average shares outstanding - basic and diluted	16,289,175	14,571,553

BIOXCEL THERAPEUTICS, INC.

STATEMENTS OF CHANGES IN STOCKHOLDERS' EQUITY (DEFICIT)

(amounts in thousands, except share and per share data)

	Common Stock		Additional Paid in	Accumulated	
	Shares	Amount	Capital	Deficit	Total
Balance as of January 1, 2018	9,907,548	$ 10	$ 3,458	$ (4,450)	$ (982)
Issuance of common stock	283,452	1	1,949	—	1,950
Issuance of common stock, upon completion of Initial Public Offering, net of issuance costs of $5,898	5,454,545	5	54,097	—	54,102
Stock-based compensation	—	—	3,082	—	3,082
Exercise of stock options	17,676	—	7	—	7
Net loss	—	—	—	(19,270)	(19,270)
Balance as of December 31, 2018	15,663,221	$ 16	$ 62,593	$ (23,720)	$ 38,889
Issuance of common stock, net of issuance costs of $1,991	2,369,223	$ 2	$ 17,808	$ —	$ 17,810
Stock-based compensation		—	3,142	—	3,142
Exercise of stock options	54,938	—	22	—	22
Net loss		—	—	(32,968)	(32,968)
Balance as of December 31, 2019	18,087,382	$ 18	$ 83,565	$ (56,688)	$ 26,895

BIOXCEL THERAPEUTICS, INC.

STATEMENTS OF CASH FLOWS

(amounts in thousands, except share and per share data)

	Year ended December 31,	
	2019	2018
CASH FLOWS FROM OPERATING ACTIVITIES:		
Net loss	$ (32,968)	$ (19,270)
Reconciliation of net loss to net cash used in operating activities		
Depreciation and amortization	156	17
Stock-based compensation expense	3,142	3,082
Changes in operating assets and liabilities:		
Prepaid expenses and other assets	(1,190)	(539)

Accounts payable, accrued expenses and other liabilities	3,580	3,201
Net cash used in operating activities	(27,280)	(13,509)
CASH FLOWS FROM INVESTING ACTIVITIES:		
Purchase of equipment	(870)	(340)
Net cash used in investing activities	(870)	(340)
CASH FLOWS FROM FINANCING ACTIVITIES:		
Proceeds from issuance of common stock, net	17,810	56,513
Exercise of options	22	7
Payable to Parent for services	—	(67)
Due to Parent	179	(555)
Note Payable — Parent	—	(371)
Net cash provided by financing activities	18,011	55,527
Net (decrease) increase in cash and cash equivalents	(10,139)	41,678
Cash and cash equivalents, beginning of the period	42,565	887
Cash and cash equivalents, end of the period	$ 32,426	$ 42,565
Supplemental cash flow information:		
Interest paid	$ 62	$ 1
Supplemental disclosure of non-cash Operating, Investing and Financing Activities:		
Deferred issuance costs reclassified to additional paid-in-capital upon completion of initial public offering	$ —	$ 461
Right-of-use asset obtained in exchange for new operating lease liability	$ 1,308	—

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