May 7, 2019

BioXcel Therapeutics Reports First Quarter 2019 Results and Provides Business Update

On track to announce results in May 2019 from recently completed BXCL501 Phase 1 pharmacokinetic (bioavailability) and safety study

Advanced manufacturing of BXCL501 thin film formulation for pivotal study

Filed IND with the FDA clinical trial of BXCL701, NKTR-214 and avelumab triple combination for treatment of pancreatic cancer

Showcased BXCL701 in combination with OX40 agonist results in late-breaking poster presentation at AACR annual meeting

BXCL501 Investor Day scheduled to be held on May 22nd, 2019 in New York City

NEW HAVEN, Conn., May 07, 2019 (GLOBE NEWSWIRE) -- BioXcel Therapeutics, Inc. ("BTI" or "Company") (Nasdaq: BTAI), today announced its quarterly results for the first quarter ended March 31, 2019 and provided an update on key strategic and operational initiatives. BTI is a clinical-stage biopharmaceutical development company utilizing novel artificial intelligence approaches to identify the next wave of medicines across neuroscience and immuno-oncology.

First Quarter 2019 and Recent Highlights:

(BXCL501)- Neuroscience Program-

- Completed dosing of multiple cohorts in Phase 1 pharmacokinetic (bioavailability) and safety study of BXCL501; data announcement expected in coming weeks
- Finalized formulation development and transitioned to automated process for manufacturing of BXCL501 thin film formulation for pivotal study
- Announced proof-of-concept data demonstrating high response rates from three independent Phase 1 studies of intravenously administered dexmedetomidine (Dex) for acute treatment of agitation in patients with schizophrenia, Alzheimer's disease / dementia and opioid withdrawal symptoms

(BXCL701)- Immuno-Oncology Program-

- Filed investigational new drug (IND) application with the U.S. Food and Drug Administration (FDA) on proposed clinical trial of BXCL701, NKTR-214 and avelumab triple combination for treatment of pancreatic cancer along with our partners Pfizer and Merck KGaA, Darmstadt, Germany, and Nektar Therapeutics
- Filed a clinical trial application (CTA) with U.K. health authorities to advance global development of BXCL701 and pembrolizumab (Keytruda®) in neuroendocrine prostate cancer (tNEPC)
- Multiple sites opened for tNEPC clinical trial and two sites selected for BXCL701 proof of mechanism study for previously opened INDs
- Presented positive preclinical data on combination of BXCL701 with OX40 agonist at the American Association for Cancer Research (AACR) Annual Meeting, showcasing synergistic anti-tumor activity and survival benefit in tumor models

Dr. Vimal Mehta, Chief Executive Officer of BTI said, "During the first quarter of 2019 a number of positive initiatives continued to drive the ongoing clinical advancement of our lead programs, BXCL501 and BXL701."

"We anticipate reporting data from our first-in-human pharmacokinetic (bioavailability) and safety study of BXCL501 in the coming weeks. The dosing of multiple cohorts has been successfully completed. We believe that these results will help lay the groundwork for launching future registration studies as well as supporting BTI's first New Drug Application (NDA) expected to be filed with the FDA in 2020. Additionally, in order to support the Phase 2/3 registration studies planned to begin in second half of 2019, the process development for BXCL501 sublingual thin film has now been transitioned to an automated robotic aided platform. We intend to employ an automated and scalable manufacturing process for clinical and commercial supply. BXCL501 has demonstrated promising potential

with intravenously administered Dex in Phase 1 trials for the acute treatment of agitation in patients with schizophrenia, Alzheimer's disease/ dementia and opioid withdrawal symptoms, where new effective therapies are desperately needed. We continue to believe that BXCL501 has the potential to be applied to a wide number of neuropsychiatric disorders and look forward to exploring opportunities as we advance development in our initial target indications.

We are pleased to report that we have filed an IND with the FDA for the proposed clinical trial of BXCL701, NKTR-214 and avelumab triple combination for treatment of pancreatic cancer with our partners Pfizer and Merck KGaA and Nektar. The triple combination therapy can potentially be effective by activating both the innate and adaptive immunity in pancreatic cancer patients. We are diligently working with our partners to progress the development of this triple combination therapy. Additionally, our ongoing Phase 1b/2 US study of BXCL701 and pembrolizumab in tNEPC is open for enrollment in multiple clinical trial sites. With the goal of expanding the clinical development of this combination globally, we have filed a CTA with U.K. health authorities. Additionally, we have selected two sites for BXCL701 proof of mechanism study in pancreatic cancer patients.

In March of this year, we presented positive findings at the AACR Annual Meeting from a preclinical study evaluating the combination of BXCL701 with an OX40 agonist. The findings demonstrated that the combination significantly elevated anti-cancer activity in tumor models compared to the control. These results also provide important validation on BXCL701's ability to stimulate both innate and adaptive immunity and we expect to further investigate the applicability of this combination."

Dr. Mehta concluded, "We continued to strengthen our management team with the appointment of industry veteran, Dr. Pascal Borderies, as the Vice President, Commercial Development and Medical Affairs who is responsible for designing and executing BTI's global commercialization and medical affairs strategy, including sales and marketing efforts. We remain intently focused on progressing our assets through clinical development throughout the remainder of the year. We believe our sound strategy, highly skilled leadership team and solid pipeline candidates make us well-positioned for continued growth in 2019 and beyond."

First Quarter 2019 Financial Results

BTI reported a net loss of $7.2 million for the first quarter of 2019, compared to a net loss of $4.3 million for the same period in 2018.

Research and development expenses were $5.6 million for the first quarter of 2019, as compared to $2.9 million for the same period in 2018. The increase was primarily due to a ramp-up of research and development costs, along with increased personnel expenses associated with BTI's two main drug candidates.

General and administrative expenses were $1.7 million for the first quarter of 2019, as compared to $1.3 million for the same period in 2018. The increase was primarily due to additional payroll and payroll-related expenses and costs associated with operating as a public company.

These results include approximately $682,000 in non-cash share -based compensation charges.

As of March 31, 2019, cash and cash equivalents totaled $36.3 million.

Conference Call:
BTI will host a conference call and webcast today at 8:30 a.m. ET. To access the call please dial (888) 394-8218 (domestic) and (323) 794-2588 (international) and provide the passcode 9482739. A live webcast of the call will be available on the Investors sections of the BTI website at www.bioxceltherapeutics.com. The archived webcast will be available through June 7, 2019.

Upcoming investor conferences:

- UBS Global Healthcare Conference - May 20-22, 2019, New York City
- BXCL501 Investor Day - May 22, 2019, New York City
- BMO Capital Markets Prescription for Success Healthcare Conference - May 25, 2019, New York City
- Jefferies Global Healthcare Conference - June 4-7, 2019, New York City

About BXCL501:
BXCL501 is a first in class, sublingual film of dexmedetomidine, a selective alpha 2a receptor agonist for the treatment of acute agitation. BTI believes that BXCL501 directly targets a causal agitation mechanism and using IV (intravenous) Dex has demonstrated anti-agitation effects in both preclinical and clinical studies. There is precedent for FDA approval and reimbursement of a non-invasive therapy for the acute treatment of agitation in patients with

schizophrenia and bipolar disease, evidenced by regulatory approval of Adasuve, an inhaled version of the antipsychotic loxapine.

About BXCL701:
BXCL701 is a first in class oral immunotherapy with dual mechanisms of action, with an established safety profile from 700 healthy subjects and cancer patients. Designed to stimulate both the innate and acquired immune systems, BXCL701 works by inhibiting dipeptidyl peptidase (DPP) 8/9 and blocking immune evasion by targeting fibroblast activation protein (FAP). Preclinical combination data evaluating BXCL701, a checkpoint inhibitor and other IO agents has demonstrated encouraging anti-tumor activity in multiple tumor types and formation of functional immunological memory. It is under development for tNEPC and pancreatic cancer.

About BioXcel Therapeutics, Inc.:
BioXcel Therapeutics, Inc. is a clinical stage biopharmaceutical company focused on drug development that utilizes novel artificial intelligence to identify the next wave of medicines across neuroscience and immuno-oncology. BTI'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. BTI's two most advanced clinical development programs are BXCL501, a sublingual thin film formulation designed for acute treatment of agitation resulting from neuropsychiatric disorders, and BXCL701, an orally administered systemic innate immunity activator designed 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, statements that relate to the advancement and development of BXCL501 and BXCL701, the commencement of clinical trials, the availability and results of data from clinical trials, BTI’s submission of its first New Drug Application with the FDA and other information that is not historical information. When used herein, words such as “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; it 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 Annual Report on Form 10-K for the fiscal year ended December 31, 2018 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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Source: BioXcel Therapeutics, Inc.

BIOXCEL THERAPEUTICS, INC.

BALANCE SHEETS

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

	March 31, 2019	December 31, 2018
	(unaudited)	
ASSETS		
Current assets		
Cash and cash equivalents	$ 36,296	$ 42,565
Prepaid expenses and other current assets	1,783	491
Due from Parent	—	115
Total current assets	38,079	43,171
Property and equipment, net	905	327
Operating lease right-of-use asset	1,293	—
Other assets	51	51
Total assets	**$ 40,328**	**$ 43,549**
LIABILITIES AND STOCKHOLDERS' EQUITY		
Current liabilities		
Accounts payable	$ 2,128	$ 1,604
Accrued expenses	3,348	3,056
Due to Parent	392	—
Other current liabilities	939	—
Total current liabilities	6,807	4,660
Operating lease liability	1,153	—
Total liabilities	**7,960**	**4,660**
Stockholders' equity		
Preferred stock, $0.001 par value, 10,000,000 shares authorized; no shares issued or outstanding	—	—
Common stock, $0.001 par value, 50,000,000 shares authorized; 15,665,802 and 15,663,221 shares issued and outstanding as of March 31, 2019 and December 31, 2018, respectively	16	16
Additional paid-in-capital	63,276	62,593
Accumulated deficit	(30,924)	(23,720)
Total stockholders' equity	32,368	38,889
Total liabilities and stockholders' equity	**$ 40,328**	**$ 43,549**

BIOXCEL THERAPEUTICS, INC.

STATEMENTS OF OPERATIONS

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

	Three Months Ended March 31,	
	2019	2018
Revenues	$ —	$ —

Operating costs and expenses		
Research and development	5,674	2,938
General and administrative	1,745	1,348
Total operating expenses	7,419	4,286
Loss from operations	(7,419)	(4,286)
Other income		
Dividend and interest income, net	215	4
Net loss	$ (7,204)	$ (4,282)
Net loss per share attributable to common stockholders basic and diluted	$ (0.46)	$ (0.37)
Weighted average shares outstanding - basic and diluted	15,663,795	11,456,325

BIOXCEL THERAPEUTICS, INC.

STATEMENTS OF CHANGES IN STOCKHOLDERS' EQUITY / DEFICIT

(amounts in thousands, except shares)
(unaudited)

	Common Stock		Additional Paid in	Accumulated	
	Shares	Amount	Capital	Deficit	Total
Balance as of December 31,2017	9,907,548	$ 10	$ 3,458	$ (4,450)	$ (982)
Issuance of common shares	283,452	1	1,949	—	1,950
Issuance of common shares, upon completion of Initial Public Offering, net of issuance costs of $5,898	5,454,545	5	54,097	—	54,102
Stock-based compensation	—	—	1,319	—	1,319
Net loss	—	—	—	(4,282)	(4,282)
Balance as of March 31, 2018	15,645,545	$ 16	$ 60,823	$ (8,732)	$ 52,107
Balance as of December 31,2018	15,663,221	$ 16	$ 62,593	$ (23,720)	$ 38,889
Stock-based compensation	—	—	682	—	682
Exercise of stock options	2,581	—	1	—	1
Net loss	—	—	—	(7,204)	(7,204)
Balance as of March 31, 2019	15,665,802	$ 16	$ 63,276	$ (30,924)	$ 32,368

BIOXCEL THERAPEUTICS, INC.

STATEMENTS OF CASH FLOWS

(amounts in thousands)
(unaudited)

	Three months ended March 31,	
	2019	2018
CASH FLOWS FROM OPERATING ACTIVITIES:		
Net loss	$ (7,204)	$ (4,282)
Reconciliation of net loss to net cash used in operating activities		
Depreciation and amortization	37	—
Stock-based compensation expense	682	1,319
Due to Parent under asset contribution agreement	500	1,000

Changes in operating assets and liabilities:		
Prepaid expenses and other assets	(1,292)	(805)
Accounts payable, accrued expenses and other	1,603	1,743
Due to related party	4	13
Net cash used in operating activities	(5,670)	(1,012)
CASH FLOWS FROM INVESTING ACTIVITIES:		
Purchases of equipment and leasehold improvements	(600)	—
Net cash used in investing activities	(600)	—
CASH FLOWS FROM FINANCING ACTIVITIES:		
Proceeds from issuance of common stock, net	—	56,512
Exercise of options	1	—
Due to Parent	—	(551)
Note Payable — Parent	—	(371)
Net cash provided by financing activities	1	55,590
Net (decrease) increase in cash and cash equivalents	(6,269)	54,578
Cash and cash equivalents, beginning of the period	42,565	887
Cash and cash equivalents, end of the period	$ 36,296	$ 55,465
Supplemental cash flow information:		
Interest paid	$ 8	$ 2
Supplemental disclosure of non-cash Financing Activity:		
Deferred issuance costs, unpaid as of December 31, 2017	$ —	$ 391
Deferred issuance costs reclassified to additional paid-in-capital upon completion of initial public offering.	$ —	$ 461
Reclassification of net Parent Investment in the Company to accumulated deficit.	$ —	$ 440

Source: BioXcel Therapeutics, Inc.