November 9, 2018

BioXcel Therapeutics Reports Third Quarter 2018 Quarterly Results and Provides Business Update

NEW HAVEN, Conn., Nov. 09, 2018 (GLOBE NEWSWIRE) -- BioXcel Therapeutics, Inc. (“BTI”) (Nasdaq: BTAI), a clinical stage biopharmaceutical development company utilizing novel artificial intelligence approaches to identify the next wave of medicines across neuroscience and immuno-oncology, today announced quarterly results for the third quarter ended September 30, 2018 and provided an update on key strategic and operational initiatives.

During the third quarter of 2018, the Company made several advances in the development of its two lead clinical programs, BXCL501, a proprietary sublingual thin film formulation of dexmedetomidine (Dex), and BXCL701, an orally-available systemic innate-immune activator.

Third Quarter 2018 and Recent Highlights:

(BXCL501)-Neuroscience Program-

- A first-in-human pharmacokinetic (bioavailability) and safety study for the sublingual thin film formulation of Dex is expected to be initiated by the end of 2018 following approval of the investigational new drug (IND) application;
  • Received valuable feedback and guidance on further development of BXCL501 during a pre-investigational new drug meeting with FDA;
  • Appointed a clinical research organization (CRO) to conduct and manage clinical studies;
  • Completed manufacturing of Company’s proprietary sublingual thin film formulation of Dex, and the drug is available for clinical studies;
- Data readout from the pharmacokinetic and safety study of BXCL501 is expected in the first half of 2019;
- Expect data readouts from intravenous (IV) Dex studies supporting BXCL501 development in acute agitation in patients with schizophrenia and senile dementia of the Alzheimer’s type (SDAT) in coming weeks;
- Established an industry leading neuro advisory board to support global development of BXCL501 and emerging neuroscience programs.

(BXCL701)-Immuno-Oncology Program-

- Received FDA acceptance of IND application for Phase 1b/2 clinical study to evaluate BXCL701 in combination with pembrolizumab (Keytruda®) in treatment emergent neuroendocrine prostate cancer (tNEPC); trial initiation is expected in fourth quarter of 2018;

  - • Completed manufacturing of BXCL701 drug product, available for clinical studies;
  - • Selected a leading CRO to support the Company in conducting and managing clinical studies;
- Data from the pharmacokinetic, safety and efficacy study of BXCL701 in tNEPC expected to be available throughout 2019;
- Entered a clinical immuno-oncology (IO) partnership with Nektar Therapeutics to develop combination of BXCL701, Nektar Therapeutics' NKTR-214 and a checkpoint inhibitor as a potential treatment for pancreatic cancer; companies will be sharing the cost of the trial;
- Established an industry leading IO clinical advisory board to support global development of BXCL701 and emerging programs.

Emerging Programs-

- Continued to use artificial intelligence platform to select and prioritize additional pipeline opportunities to augment the current neuroscience and IO portfolio.

Business & Operations-

- Strengthened management team with drug development experts including:
  - • Chetan Lathia, Ph.D. as Senior Vice President and Head of Translational Medicine, Clinical Pharmacology and Regulatory Affairs, to support advancement of the pipeline programs.
  - • David Hanley, Ph.D. as Vice President and Head of Global Pharmaceutical Development and Operations, to lead the pharmaceutical development activities and operational efforts.

Vimal Mehta, Ph.D., President and Chief Executive Officer of BTI, commented, "We have made tremendous progress during the past quarter that we believe is truly transformational for BTI. We are well positioned to execute on our clinical programs across our two primary areas of focus."

"In September, we received FDA IND clearance for our first-in-human Phase 1b/2 trial to evaluate BXCL701 in combination with Keytruda® as a potential therapy for tNEPC. We anticipate initiating this clinical study prior to year-end. In addition, we expanded our research collaboration with Nektar Therapeutics into a new clinical partnership for further development of the triple combination of BXCL701, Nektar Therapeutics' NKTR-214 and a checkpoint inhibitor. Today, we are presenting encouraging preclinical data on the triple combination across multiple tumor models at the 2018 Society for Immunotherapy of Cancer (SITC) Annual Meeting. This study demonstrated robust anti-tumor activity resulting in complete tumor regression in certain in vivo models and generation of functional immunological memory, supporting the triple combination as a potential therapy for pancreatic cancer and other tumors."

"Following our productive interactions with the FDA on BXCL501, we plan to initiate our first-in-human pharmacokinetic (bioavailability) and safety trial once our IND is approved. The aim of this trial is to define the optimal dose of BXCL501 and collect sufficient clinical data to proceed to a Phase 3 registration trial in 2019. We have previously reported positive data from our trial of IV Dex, which were essential in determining the ideal dosing strategy. We now expect that data from the IV Dex studies in patients with schizophrenia and SDAT will

be available by the end of 2018."

"We are de-risking clinical development of our lead candidates through patient selection optimization, translational research and predictive biomarker discovery utilizing artificial intelligence approaches. Further, we continue to identify additional opportunities for BXCL501 and BXCL701 and plan to pursue their development. We anticipate filing both INDs and clinical trial applications for additional indications across multiple locations, in an effort to establish our global footprint and leverage the significant value of our lead clinical programs."

Dr. Mehta concluded, "We remain firmly committed to our goal of providing patients with transformative therapies while also creating value for our shareholders. We are very excited by what the future holds for our company and look forward to delivering on the anticipated milestones."

Third Quarter 2018 Financial Results

BTI reported a net loss of $4.9 million for the third quarter of 2018, compared to a net loss of $0.9 million for the same period in 2017.

Research and development expenses were $3.8 million for the third quarter of 2018, as compared to $0.6 million for the same period in 2017. The increase was primarily due to an expansion of research and development activities, including increased personnel costs, professional fees, clinical trials, and manufacturing costs associated with BTI's two lead drug candidates.

General and administrative expenses were $1.3 million for the third quarter of 2018, as compared to $0.3 million for the same period in 2017. The increase was primarily due to additional payroll and payroll-related expenses, professional fees and costs associated with operating as a public company.

As of September 30, 2018, cash and cash equivalents totaled $47.1 million.

Upcoming investor conferences:

- Jefferies Global Healthcare Conference, November 14-15, 2018, London
- 2018 Prescriptions for Success Healthcare Conference, December 12, 2018, New York
- Investor access event at the J.P. Morgan Healthcare Conference, January 7-10, 2019, San Francisco

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 has demonstrated anti-agitation effects in preclinical and clinical studies. It has a well-established regulatory and reimbursement path in schizophrenia and bipolar disorder, as demonstrated by a previously-approved drug, Adasuve.

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 approaches 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's two most advanced clinical development programs are BXCL501, a sublingual thin film formulation designed for acute treatment of agitation resulting from neurological and psychiatric disorders, and BXCL701, an immuno-oncology agent designed for treatment of a rare form of prostate cancer and for treatment of pancreatic cancer.

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 of data from clinical trials 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 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, market conditions and the factors described under the caption “Risk Factors” in BioXcel’s Form 10-Q for the quarter ended September 30, 2018, and BioXcel’s other filings made with the Securities and Exchange Commission. Consequently, forward-looking statements should be regarded solely as BioXcel’s current plans, estimates and beliefs. Investors should not place undue reliance on forward-looking statements. BioXcel cannot guarantee future results, events, levels of activity, performance or achievements. BioXcel does not undertake and specifically declines any obligation to update, republish, or revise any forward-looking statements to reflect new information, future events or circumstances or to reflect the occurrences of unanticipated events, except as may be required by law.

Contact Information:
The Ruth Group for BTI:
Lee Roth / Janhavi Mohite
646-536-7012 / 7026

lroth@theruthgroup.com / jmohite@theruthgroup.com

BIOXCEL THERAPEUTICS, INC.

BALANCE SHEETS

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

	September 30, 2018 (unaudited)	December 31, 2017
ASSETS		
Current assets		
Cash and cash equivalents	$ 47,122	$ 887
Prepaid expenses and other current assets	467	3
Due from Parent	49	—
Total current assets	47,638	890
Deferred offering expenses	—	461
Equipment, net	177	4
Other assets	51	—
Total assets	**$ 47,866**	**$ 1,355**
LIABILITIES AND STOCKHOLDERS' EQUITY (DEFICIT)		
Current liabilities		
Accounts payable	$ 1,213	$ 444
Accrued expenses	830	1,015
Payable to Parent for services	—	67
Note payable to Parent	—	371
Due to Parent	—	440
Total current liabilities	2,043	2,337
Total liabilities	**2,043**	**2,337**
Stockholders' equity (deficit)		
Preferred stock, $0.001 par value, 10,000,000 shares authorized; no shares issued or outstanding	—	—
Common stock, $0.001 par value, 50,000,000 shares authorized; 15,645,545 and 9,907,548 shares issued and outstanding as of September 30,2018 and December 31, 2017, respectively	16	10
Additional paid-in-capital	62,452	3,458
Accumulated deficit	(16,645)	(4,450)
Total stockholders' equity (deficit)	45,823	(982)
Total liabilities and stockholders' equity (deficit)	**$ 47,866**	**$ 1,355**

BIOXCEL THERAPEUTICS, INC.

STATEMENTS OF OPERATIONS

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2018	**2017**	**2018**	**2017**
Revenues	$ —	$ —	$ —	$ —
Operating costs and expenses				
Research and development	3,821	619	8,540	1,264
General and administrative	1,298	298	4,109	747
Total operating expenses	5,119	917	12,649	2,011
Loss from operations	(5,119)	(917)	(12,649)	(2,011)
Other income				
Dividend and interest income, net	232	—	454	—
Net loss	$ (4,887)	$ (917)	$ (12,195)	$ (2,011)
Net loss per share attributable to common stockholders/ Parent basic and diluted	$ (0.31)	$ (0.10)	$ (0.86)	$ (0.21)
Weighted average shares outstanding - basic and diluted	15,645,545	9,483,318	14,228,192	9,483,318

BIOXCEL THERAPEUTICS, INC.

STATEMENTS OF CASH FLOWS

(amounts in thousands)
(unaudited)

	Nine months ended September 30,	
	2018	**2017**
CASH FLOWS FROM OPERATING ACTIVITIES:		

Net loss	$ (12,195)	$ (2,011)
Reconciliation of net loss to net cash used in operating activities		
Depreciation and amortization	9	1
Stock-based compensation expense	2,949	516
Changes in operating assets and liabilities:		
Prepaid expenses and other assets	(515)	(9)
Accounts payable and accrued expenses	584	405
Net cash used in operating activities	(9,168)	(1,098)
CASH FLOWS FROM INVESTING ACTIVITIES:		
Purchase of equipment	(182)	—
Net cash used in investing activities	(182)	—
CASH FLOWS FROM FINANCING ACTIVITIES:		
Proceeds from issuance of common stock, net	56,512	751
Net Parent Investment	—	214
Payable to Parent for services	(67)	88
Due to Parent	(489)	430
Note Payable — Parent	(371)	369
Net cash provided by financing activities	55,585	1,852
Net increase in cash and cash equivalents	46,235	754
Cash and cash equivalents, beginning of the period	887	—
Cash and cash equivalents, end of the period	$ 47,122	$ 754
Supplemental cash flow information:		
Interest paid	$ 1	$ —
Supplemental disclosure of non-cash Financing Activity:		
Deferred issuance costs reclassified to additional paid-in-capital upon completion of initial public offering.	$ 461	$ —

Source: BioXcel Therapeutics, Inc.