

August 8, 2018



BioXcel Therapeutics Reports Second Quarter 2018 Financial Results and Provides Business Update

Clinical programs in neuroscience (BXCL501) and immuno-oncology (BXCL701) on track

Company strengthens business operations and infrastructure

NEW HAVEN, Conn., Aug. 08, 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 financial results for the second quarter ended June 30, 2018, and provided an update on key strategic and operational initiatives. During the second quarter, the Company made continued progress in the development of its two lead clinical programs. BXCL501, a proprietary sublingual thin film formulation of dexmedetomidine (Dex), is being developed for the acute treatment of mild to moderate agitation in neurological and psychiatric disorders such as geriatric dementia and schizophrenia/bipolar disorder. BXCL701, a first-in-class oral small-molecule immunomodulator targeting dipeptyl peptidases 8/9 (DPP 8/9) and fibroblast activation protein (FAP) is being developed for treatment emergent neuroendocrine prostate cancer (tNEPC) and pancreatic cancer.

Second Quarter 2018 and Recent Highlights

Neuroscience Program (BXCL501)-

- Reported positive results from Phase 1b study of intravenously dosed Dex validating clinical development strategy for BXCL501;
- Data supports potential dosing strengths for sublingual thin film formulation of BXCL501, and initiation of Phase 1b PK/PD safety study using IV Dex in mild probable Alzheimer's (AD) patients;
- Company plans to initiate Phase 1b PK/PD safety study using the IV formulation of Dex in schizophrenia patients in the second half of 2018;
- Data from both AD and schizophrenia studies expected during the second half of 2018;
- Initiation of bioavailability study for the sublingual thin film formulation expected in the second half of 2018 following completion of GMP manufacturing and IND approval;
- Formed world-class neuroscience clinical advisory board to support global development of BXCL501 and other neuroscience programs;
- Maintained active dialog with FDA regarding ongoing clinical development of BXCL501.

Immuno-Oncology Program (BXCL701)-

- Company plans to commence single and combination agent open label clinical trials of BXCL701 in the second half of 2018 following completion of GMP manufacturing and IND approval. Preliminary data from these trials will be available throughout 2019;
- Presented encouraging preclinical data on combination therapy of BXCL701, Nektar Therapeutics' NKTR-214 and an anti-PD1 agent in pancreatic cancer at the 2018 ASCO Annual Meeting.

Business & Operations-

- Strengthened management team with C-suite and VP-level appointments;
- Formalized the role of Vincent O'Neill MD, as Senior Vice President and Chief Medical Officer;
- Key hires included Vikas Sharma, Ph.D. as Vice President of Business Development to lead the Company's strategic partnering initiatives, particularly for BTI's lead immuno-oncology asset, BXCL701; Cedric Burg, Ph.D. as Vice President and Head of Global Clinical Operations and Project Management to lead the development and execution of BTI's global clinical operations strategy; and Michael DeVivo Ph.D. as Vice President, Neuroscience, to support the Company's research and development team in advancing current programs and expanding pipeline candidates using the dual strategy of focusing on symptoms-based treatments, and treatments for rare neurological diseases;
- Continued expanding network of partners to leverage capabilities and augment internal drug development teams;
- Continued to build intellectual property portfolio with multiple global patent submissions to protect lead candidates, BXCL501 and BXCL701.

"During the second quarter, we made tremendous progress advancing our lead neuroscience and immuno-oncology programs with positive results from two important studies, as well as further building out our leadership infrastructure," commented Vimal Mehta, President and Chief Executive Officer of BTI.

"We reported positive data from our first-in-human trial of intravenously (IV)-dosed Dex supporting further development of BXCL501, our sublingual formulation of Dex for the acute treatment of agitation. Data from the trial demonstrated that the doses and exposure levels of IV Dex had calming effects on healthy subjects without producing clinically meaningful changes in blood pressure or heart rate. The results from this trial will be valuable in determining the ideal dosing strategy for BXCL501. Based on our ongoing discussions with the FDA regarding development of BXCL501, we expect that proof-of-concept studies in geriatric dementia and schizophrenia/bipolar disease will be initiated in the second half of 2018. We also expect to initiate a bioavailability study on our proprietary sublingual thin film formulation this year."

Dr. Mehta added, "At the American Society of Clinical Oncology (ASCO) 2018 annual meeting, we showcased promising preclinical data on a combination of BXCL701, our oral small molecule immunomodulator, Nektar Therapeutics' NKTR-214 and an anti-PD1 agent in pancreatic cancer models. The study showed durable anti-tumor response with

significant improvement in complete tumor regression and development of anti-cancer immunity. Recently, one component of BXCL701's dual mechanism of action, DPP 8/9 inhibition, was highlighted in peer reviewed journal, *Nature Medicine*, providing a key validation of our choice of lead immuno-oncology program and clinical strategy. We are now preparing to initiate clinical studies for BXCL701 in tNEPC and pancreatic cancer in the second half of the year."

Dr. Mehta concluded, "We are well positioned as we enter the third quarter of the year, with a strong cash runway of over \$50M to develop our two drug candidates. We have established our leadership team with the appointment of renowned executives who bring international pharma expertise to the company, especially in neuroscience and immuno-oncology. We believe we are well-equipped to build upon the progress we have made in the second quarter of 2018 to achieve our operational and clinical development goals. We remain committed to working towards discovery and development of innovative therapeutic options for neuroscience and immuno-oncology indications, while also creating value for our shareholders.

Second Quarter 2018 Financial Results

BTI reported a net loss of \$3.0 million for the second quarter of 2018, compared to a net loss of \$0.6 million for the same period in 2017.

Research and development expenses were \$1.8 million for the second quarter of 2018, as compared to \$0.3 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 trial, and manufacturing costs all, associated with BTI's two main drug candidates.

General and administrative expenses were \$1.5 million for the second quarter of 2018, as compared to \$0.2 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 June 30, 2018, cash and cash equivalents totaled \$50.3 million.

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. 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. The Company'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 June 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.

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BIOXCEL THERAPEUTICS, INC.

BALANCE SHEETS

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

	<u>June 30, 2018</u>	<u>December 31, 2017</u>
	(unaudited)	
ASSETS		
Current assets		
Cash and cash equivalents	\$ 50,349	\$ 887
Prepaid expenses and other current assets	669	3
Due from Parent	70	—
Total current assets	<u>51,088</u>	<u>890</u>

Deferred offering expenses	—	461
Equipment, net	122	4
Total assets	\$ 51,210	\$ 1,355

LIABILITIES AND STOCKHOLDERS' EQUITY (DEFICIT)

Current liabilities		
Accounts payable	\$ 889	\$ 444
Accrued expenses	500	1,015
Payable to Parent for services	—	67
Note payable to Parent	—	371
Due to Parent	—	440
Total current liabilities	1,389	2,337
Total liabilities	1,389	2,337

Commitments and contingencies	—	—
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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 June 30, 2018 and December 31, 2017, respectively	16	10
Additional paid-in-capital	61,563	3,458
Accumulated deficit	(11,758)	(4,450)
Total stockholders' equity (deficit)	49,821	(982)
Total liabilities and stockholders' equity (deficit)	\$ 51,210	\$ 1,355

BIOXCEL THERAPEUTICS, INC.
STATEMENTS OF OPERATIONS
(amounts in thousands, except shares and per share data)
(unaudited)

	Three Months Ended		Six Months Ended	
	June 30,		June 30,	
	2018	2017	2018	2017
Revenues	\$ —	\$ —	\$ —	\$ —
Operating costs and expenses				
Research and development	1,781	324	4,719	645
General and administrative	1,463	241	2,811	449
Total operating expenses	3,244	565	7,530	1,094
Loss from operations	(3,244)	(565)	(7,530)	(1,094)
Other income				
Dividend and interest income, net	218	—	222	—

Net loss	<u>\$ (3,026)</u>	<u>\$ (565)</u>	<u>\$ (7,308)</u>	<u>\$ (1,094)</u>
Net loss per share attributable to common stockholders/Parent basic and diluted	<u>\$ (0.19)</u>	<u>\$ (0.06)</u>	<u>\$ (0.54)</u>	<u>\$ (0.12)</u>
Weighted average shares outstanding - basic and diluted	15,645,545	9,480,000	13,507,770	9,480,000

BIOXCEL THERAPEUTICS, INC.
STATEMENTS OF CASH FLOWS
(amounts in thousands)
(unaudited)

	<u>Six months ended June 30,</u>	
	<u>2018</u>	<u>2017</u>
CASH FLOWS FROM OPERATING ACTIVITIES:		
Net loss	\$ (7,308)	\$ (1,094)
Reconciliation of net loss to net cash used in operating activities		
Depreciation and amortization	2	—
Stock-based compensation expense	2,060	200
Changes in operating assets and liabilities:		
Prepaid expenses	(666)	2
Accounts payable and accrued expenses	(70)	116
Net cash used in operating activities	<u>(5,982)</u>	<u>(776)</u>
CASH FLOWS FROM INVESTING ACTIVITIES:		
Purchase of equipment	(120)	—
Net cash used in investing activities	<u>(120)</u>	<u>—</u>
CASH FLOWS FROM FINANCING ACTIVITIES:		
Proceeds from issuance of common stock, net	56,512	—
Net Parent Investment	—	214
Payable to Parent for services	(67)	—
Due to Parent	(510)	562
Note Payable - Parent	(371)	285
Net cash provided by financing activities	<u>55,564</u>	<u>1,061</u>
Net increase in cash and cash equivalents	49,462	285
Cash and cash equivalents, beginning of the period	887	—
Cash and cash equivalents, end of the period	<u>\$ 50,349</u>	<u>\$ 285</u>

Supplemental cash flow information:

Interest paid	\$	1	—
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Supplemental disclosure of non-cash Financing Activity:

Deferred issuance costs reclassified to additional paid-in-capital upon completion of initial public offering.	\$	461	—
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Source: BioXcel Therapeutics, Inc.