

March 7, 2019



BioXcel Therapeutics Reports Fourth Quarter and Full Year 2018 Quarterly Results and Provides Business Update

BXCL501 Pharmacokinetic (Bioavailability) and safety study on track to read out in second quarter 2019; initiation of Phase 2/3 Registration Trial expected in second half 2019

Human Proof of Concept established in multiple indications for BXCL501, high response rates observed with IV Dex in Schizophrenia, Alzheimer's Disease / Dementia and Opioid Withdrawal Patients

Clinical partnership established with Pfizer and Merck KGaA, Darmstadt, Germany for development of triple combination therapy with BXCL701 for pancreatic cancer

Three INDs accepted by FDA for lead clinical programs and Fast Track designation for BXCL501 granted in fourth quarter 2018; validation of AI-based approach to drug development

NEW HAVEN, Conn., March 07, 2019 (GLOBE NEWSWIRE) -- BioXcel Therapeutics, Inc. ("BTI" or "Company") (Nasdaq: BTAI), today announced quarterly results for the fourth quarter and full year ended December 31, 2018 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.

Fourth Quarter 2018 and Recent Highlights:

(BXCL501)-Neuroscience Program-

- Dosed multiple patient cohorts in the first-in-human pharmacokinetic (bioavailability) and safety study of BXCL501, a novel sublingual thin film formulation of dexmedetomidine (Dex)
- Received FDA Fast Track Designation for BXCL501 for acute treatment of mild to moderate agitation associated with schizophrenia, bipolar disorder, or dementia
- Reported positive proof-of-concept data from two independent Phase 1 studies of intravenously administered Dex with high response rates in Schizophrenia and Alzheimer's Disease / Dementia, supporting BXCL501 as a potential therapy for acute treatment of agitation in these indications
- Expanded indication for BXCL501 to treat symptoms associated with opioid withdrawal based on positive data from a third Phase 1b study of IV Dex

(BXCL701)-Immuno-Oncology Program-

- Received FDA acceptance of IND for BXCL701, an orally-available systemic innate-

immune activator that inhibits dipeptidyl peptidase (DPP) 8/9 and fibroblast activation protein (FAP), for treatment emergent neuroendocrine prostate cancer (tNEPC); patient recruitment ongoing

- Received FDA IND acceptance for clinical trial of BXCL701 in pancreatic cancer to advance understanding of its mechanism of action (MoA); clinical site being activated
- Established clinical partnership with Pfizer and Merck KGaA, Darmstadt, Germany, to evaluate BXCL701 combination therapy in pancreatic cancer, along with Nektar Therapeutics
- Received positive feedback from FDA pre-IND meeting on proposed clinical trial of BXCL701, NKTR-214 and avelumab for treatment of metastatic pancreatic cancer
- Continued to explore additional combination therapy approaches to expand BXCL701's target indications beyond tNEPC and pancreatic cancer

Emerging Programs-

- Continued to leverage the artificial intelligence platform owned by BioXcel Corporation, BTI's parent, to select and prioritize additional development opportunities to expand the current portfolio and broaden the addressable market for its lead programs through identification of new indications

Dr. Vimal Mehta, Chief Executive Officer of BTI said, "Following our successful Initial Public Offering we achieved significant clinical, regulatory and operational milestones throughout 2018. Over the last 12 months, we have initiated multiple human clinical trials for BXCL501 and BXCL701, reported positive human proof-of-concept data from several trials, signed new clinical partnerships, significantly advanced Chemistry, Manufacturing and Controls (CMC) work on our lead programs and expanded the addressable markets for these programs. In addition, we are in discussions with a number of highly qualified Board candidates and anticipate appointing a new board member in the near future. As we evolve to a late-clinical-stage organization, we remain confident in the potential of our pipeline assets to generate meaningful clinical benefit for patients in need.

"We dosed multiple patient cohorts in our first-in-human pharmacokinetic (bioavailability) and safety study of BXCL501, and we are on track to report data from this study in the first half of 2019. This data will help establish a foundation to launch registration studies later this year. Additionally, we received FDA Fast Track designation for BXCL501 for the acute treatment of mild to moderate agitation associated with schizophrenia, bipolar disorder, or dementia. This regulatory designation would potentially facilitate the development of BXCL501, allow more frequent meetings and more frequent written communication with the FDA, and expedite its regulatory review.

"In the fourth quarter of 2018, we reported positive data from the Phase 1 studies of Dex for acute treatment of agitation in Schizophrenia and Senile Dementia of the Alzheimer's Type (SDAT) patients. The positive data from these trials support the continued clinical development of BXCL501 for the acute treatment of agitation in schizophrenia and dementia. We also recently announced positive data from a Phase 1b trial that established the potential application of BXCL501 for the treatment of opioid withdrawal symptoms.

"In our BXCL701 program, we received IND acceptances from the FDA to commence trials in both tNEPC and pancreatic cancer. The Phase 1b trial, which was initiated in late 2018 for tNEPC, will be conducted in combination with pembrolizumab (Keytruda®) and will examine

safety, pharmacokinetics and anti-tumor activity of the combination therapy. Data from this trial is expected throughout 2019. We also plan to initiate a clinical study to understand the underlying role of BXCL 701 and its mechanism of action in treatment of pancreatic cancer. Pfizer and Merck KGaA recently joined our clinical collaboration with Nektar to advance the triple combination of BXCL701, NKTR-214 and avelumab in this indication. This collaboration reinforces the industry's enthusiasm around the potential of BXCL701 to treat pancreatic cancer. We believe that our triple combination therapy has the ability to target multiple facets of the disease etiology as well as activate the immune system to produce a clinical benefit in pancreatic cancer patients. We are highly encouraged by this collaboration and look forward to leveraging the regulatory and clinical expertise of our partners as we move forward with our development plans."

Dr. Mehta concluded, "In an effort to further expand the addressable indications for BXCL701, we continue to explore additional combination therapy approaches beyond tNEPC and pancreatic cancer. We are pleased to announce that a recent preclinical study of BXCL701 in combination with an OX40-Agonist was accepted as a late-breaking abstract at the upcoming AACR Annual Meeting, which we believe demonstrates the broader potential of our lead immuno-oncology candidate beyond tNEPC and pancreatic cancer.

"We are extremely pleased with the substantial progress we have made in advancing both BXCL501 and BXCL701, and believe that our recent achievements have positioned us well for continued growth in 2019 and beyond."

Fourth Quarter & Full Year 2018 Financial Results

BTI reported a net loss of \$7.1 million for the fourth quarter of 2018, compared to a net loss of \$2.5 million for the same period in 2017.

Research and development expenses were \$6.0 million for the fourth quarter of 2018, as compared to \$1.4 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 fourth quarter of 2018, as compared to \$1.1 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.

BTI reported a net loss of \$19.3 million for the full year 2018, compared to a net loss of \$4.5 million for the same period in 2017.

Research and development expenses were \$14.5 million for full year 2018, as compared to \$2.7 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 \$5.4 million for full year 2018, as compared to \$1.8 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 December 31, 2018, cash and cash equivalents totaled \$42.6 million.

Upcoming investor conferences:

- 39th Annual Cowen Healthcare Conference – March 11-13, 2019, Boston
- Barclays Global Healthcare Conference – March 12-14, 2019, Miami
- Oppenheimer & Co. 29th Annual Healthcare Conference – March 19-20, 2019, New York City
- H.C. Wainwright Global Life Sciences Conference – April 7-9, 2019, London
- ThinkEquity Conference – May 2, 2019, New York City
- UBS Conference – May 20-22, 2019, New York City
- BMO Capital Markets Prescription for Success Healthcare Conference – May 25, 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 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 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. 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 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 other risks detailed from time to time in BTI’s Securities and Exchange Commission filings. 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 share and per share data)

	December 31, 2018	December 31, 2017
	<hr/>	<hr/>
ASSETS		
Current assets		
Cash and cash equivalents	\$ 42,565	\$ 887
Prepaid expenses and other current assets	491	3
Due from Parent	115	—
Total current assets	<hr/> 43,171	<hr/> 890
Deferred offering expenses	—	461
Equipment, net	327	4
Other assets	51	—
	<hr/>	<hr/>

Total assets	\$ 43,549	\$ 1,355
LIABILITIES AND STOCKHOLDERS' EQUITY (DEFICIT)		
Current liabilities		
Accounts payable	\$ 1,604	\$ 444
Accrued expenses	3,056	1,015
Payable to Parent for services	—	67
Note payable to Parent	—	371
Due to Parent	—	440
Total current liabilities	<u>4,660</u>	<u>2,337</u>
Total liabilities	<u>4,660</u>	<u>2,337</u>
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; 15,663,221 and 9,907,548 shares issued and outstanding as of December 31, 2018 and December 31, 2017, respectively	16	10
Additional paid-in-capital	62,593	3,458
Accumulated deficit	(23,720)	(4,450)
Total stockholders' equity (deficit)	<u>38,889</u>	<u>(982)</u>
Total liabilities and stockholders' equity (deficit)	<u>\$ 43,549</u>	<u>\$ 1,355</u>

BIOXCEL THERAPEUTICS, INC

STATEMENTS OF OPERATIONS

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

	2018	2017
Revenues	<u>\$ —</u>	<u>\$ —</u>
Operating costs and expenses		
Research and development	14,558	2,690
General and administrative	5,404	1,847
Total operating expenses	<u>19,962</u>	<u>4,537</u>
Loss from operations	(19,962)	(4,537)
Other income		
Dividend and interest income, net	692	(2)
Net loss	<u>\$ (19,270)</u>	<u>\$ (4,539)</u>

Net loss per share attributable to common stockholders/ Parent basic and diluted	\$ (1.32)	\$ (0.47)
Weighted average shares outstanding - basic and diluted	14,571,553	9,685,005

BIOXCEL THERAPEUTICS, INC
STATEMENTS OF CHANGES IN STOCKHOLDERS' EQUITY (DEFICIT)/NET PARENT
INVESTMENT

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

	Common Stock		Net Parent Investment	Additional Paid in Capital	Accumulated Deficit	Total
	Shares	Amount				
Balance as of January 1, 2017	—	\$ —	(324)	—	—	(324)
Investment from Parent	—	—	539	—	—	539
Net loss	—	—	(529)	—	—	(529)
Balance as of March 29, 2017 (date of incorporation)	—	—	(314)	—	—	(314)
Issuance of common shares	9,907,548	10	—	2,051	—	2,061
Liabilities assumed from Parent	—	—	(126)	—	—	(126)
Transfer to accumulated deficit	—	—	440	—	(440)	—
Stock-based compensation	—	—	—	1,407	—	1,407
Net loss	—	—	—	—	(4,010)	(4,010)
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	—	—	—	3,082	—	3,082
Exercise of stock options	17,676	—	—	7	—	7
Net loss	—	—	—	—	(19,270)	(19,270)
Balance as of December 31, 2018	<u>15,663,221</u>	<u>\$ 16</u>	<u>\$ —</u>	<u>\$ 62,593</u>	<u>\$ (23,720)</u>	<u>\$ 38,889</u>

BIOXCEL THERAPEUTICS, INC

STATEMENTS OF CASH FLOWS

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

	<u>Year ended December 31,</u>	
	<u>2018</u>	<u>2017</u>
CASH FLOWS FROM OPERATING ACTIVITIES:		
Net loss	\$ (19,270)	\$ (4,539)
Reconciliation of net loss to net cash used in operating activities		
Depreciation and amortization	17	1
Stock-based compensation expense	3,082	1,606
Changes in operating assets and liabilities:		
Prepaid expenses and other assets	(539)	(1)
Accounts payable and accrued expenses	3,201	737
Net cash used in operating activities	<u>(13,509)</u>	<u>(2,196)</u>
CASH FLOWS FROM INVESTING ACTIVITIES:		
Purchase of equipment	(340)	—
Net cash used in investing activities	<u>(340)</u>	<u>—</u>
CASH FLOWS FROM FINANCING ACTIVITIES:		
Proceeds from issuance of common stock, net	56,513	2,061
Exercise of options	7	—
Net Parent Investment	—	214
Deferred offering expense	—	(70)
Payable to Parent for services	(67)	67
Due to Parent	(555)	440
Note Payable — Parent	(371)	371
Net cash provided by financing activities	<u>55,527</u>	<u>3,083</u>

Net increase in cash and cash equivalents	41,678	887
Cash and cash equivalents, beginning of the period	887	—
Cash and cash equivalents, end of the period	<u>\$ 42,565</u>	<u>\$ 887</u>
Supplemental cash flow information:		
Interest paid	\$ 1	\$ —
Supplemental disclosure of non-cash Financing Activities:		
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.