November 14, 2019

BioXcel Therapeutics Reports Third Quarter 2019 Financial Results and Provides Business Update

Key data readouts expected for BXCL501 in 1H 2020—including results of pivotal Phase 3 trials—with cash position sufficient through completion of studies

Phase 1b/2 trial for geriatric dementia/Alzheimer's patients on track to readout in 1H 2020

Safety data readouts expected for BXCL701 Phase 1b/2 trial in treatment emergent Neuroendocrine Prostate Cancer (tNEPC) in Q4 2019; efficacy data readouts expected in 1H 2020

NEW HAVEN, Conn.--(BUSINESS WIRE)-- 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 third quarter ended September 30, 2019 and provided an update on key strategic and operational initiatives.

"We are pleased with the progress made on both of our clinical development programs during the quarter," stated Vimal Mehta, Chief Executive Officer of BTAI. "BXCL501, our candidate for the acute treatment of agitation in patients with schizophrenia and bipolar disease and delivered in a sublingual thin film, is highly differentiated from the current standards of care, which can produce unwanted side effects and be difficult for caregivers to administer. BXCL501 has the potential to significantly improve care for patients, while providing healthcare providers with an important new option for treating their patients. We're looking forward to reporting pivotal data during the first half of 2020."

Dr. Mehta added, "We are also pleased with the progress of our Phase 1b/2 double combination study of BXCL701 and Keytruda for tNEPC and anticipate additional safety data readouts from both cohorts in the fourth quarter of this year, followed by expected initial efficacy data in the first half of 2020."

Third 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.

- BXCL501 met its primary endpoint and demonstrated statistically significant mean reduction in PEC (PANSS, or the Positive and Negative Syndrome Scale, Excitatory Component) in the Phase 1b trial with agitated schizophrenia patients. Pivotal studies for the acute treatment of agitation in schizophrenia and bipolar patients are expected to initiate in Q4 2019, with data readouts expected 1H 2020;
- The Phase 1b/2 study of BXCL501 for acute treatment of agitation in geriatric dementia/Alzheimer's disease is expected to begin in Q4 2019, with data expected in 1H 2020;
- Initiated BXCL501 strategic initiative to investigate the feasibility of development of digital device technology, such as the Apple Watch, that can be used in conjunction with BXCL501 to enhance the prevention and treatment of agitation, specifically in geriatric dementia patients;
- Awarded grant by CDMRP to expand clinical development of BXCL501 program for the treatment of alcohol and substance abuse disorders related to PTSD in collaboration with Yale University.

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.

- The Phase 1b/2 trial of BXCL701 and Keytruda for tNEPC is ongoing. The Company presented safety and tolerability data from the first patient cohort at the Annual Prostate Cancer Foundation Scientific Retreat and is currently enrolling a second patient cohort. BTI expects to report additional safety findings by year-end before advancing to the Phase 2 stage of the trial;

- BXCL701 received third orphan drug designation (ODD) from the FDA for the treatment of AML. Potential to expand clinical development of BXCL701 program into hematological malignancies;
- 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

- BioXcel raised gross proceeds of $19.0 million in the quarter, the net of which, together with current reserves, provides sufficient capital to fund operations through key data readouts including Phase 3 and Phase 1b/2 studies with BXCL501.

Third Quarter 2019 Financial Results

BTI reported a net loss of $9.0 million for the third quarter of 2019, compared to a net loss of $4.9 million for the same period in 2018. The third quarter 2019 results include approximately $0.8 million in non-cash stock based compensation.

Research and development expenses were $7.1 million for the third quarter of 2019, as compared to $3.8 million for the same period in 2018. The increase was primarily due to an expansion of research and development activities, including increased personnel costs, clinical trials expenses, and professional fees, associated with BTI's two lead product candidates.

General and administrative expenses were $2.0 million for the third 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, professional fees and costs associated with operating as a public company.

As of September 30, 2019, cash and cash equivalents totaled approximately $40.3 million which included proceeds from the Company's follow-on-financing completed on September 30, 2019. BTI believes it is well positioned to execute on key milestones.

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 November 28, 2019.

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 wearable digital device initiative 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; 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 Quarterly Report on Form 10-Q for the quarterly period ended September 30, 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)

	September 30, 2019	December 31, 2018
	(unaudited)	
ASSETS		
Current assets		
Cash and cash equivalents	$ 40,252	$ 42,565
Prepaid expenses and other current assets	1,109	491
Due from Parent	—	115
Total current assets	41,361	43,171
Property and equipment, net	1,086	327
Operating lease right-of-use asset	1,199	—
Other assets	51	51
Total assets	**$ 43,697**	**$ 43,549**
LIABILITIES AND STOCKHOLDERS' EQUITY		
Current liabilities		
Accounts payable	$ 4,238	$ 1,604
Accrued expenses	3,387	3,056
Due to Parent	59	—
Other current liabilities	522	—
Total current liabilities	8,206	4,660
Operating lease liability	1,071	—
Total liabilities	**9,277**	**4,660**
Stockholders' equity		
Preferred stock, $0.001 par value, 10,000,000 shares authorized; no shares issued or outstanding	—	—

Common stock, $0.001 par value, 50,000,000 shares authorized; 18,035,025 and 15,663,221 shares issued and outstanding as of September 30, 2019 and December 31, 2018, respectively	18	16
Additional paid-in-capital	82,815	62,593
Accumulated deficit	(48,413)	(23,720)
Total stockholders' equity	34,420	38,889
Total liabilities and stockholders' equity	**$ 43,697**	**$ 43,549**

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2019	**2018**	**2019**	**2018**
Revenues	$ —	$ —	$ —	$ —
Operating costs and expenses				
Research and development	7,122	3,821	19,302	8,540
General and administrative	2,012	1,298	5,886	4,109
Total operating expenses	9,134	5,119	25,188	12,649
Loss from operations	(9,134)	(5,119)	(25,188)	(12,649)
Other income				
Dividend and interest income, net	116	232	495	454
Net loss	$ (9,018)	$ (4,887)	$ (24,693)	$ (12,195)
Net loss per share attributable to common stockholders basic and diluted	$ (0.57)	$ (0.31)	$ (1.57)	$ (0.86)
Weighted average shares outstanding - basic and diluted	15,752,196	15,645,545	15,695,263	14,228,192

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
Stock-based compensation	—	—	740	—	740
Net loss	—	—	—	(3,026)	(3,026)
Balance as of June 30, 2018	15,645,545	$ 16	$ 61,563	$ (11,758)	$ 49,821
Stock-based compensation	—	—	889	—	889
Net loss	—	—	—	(4,887)	(4,887)
Balance as of September 30, 2018	15,645,545	$ 16	$ 62,452	$ (16,645)	$ 45,823
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
Issuance of common shares, net of issuance costs of $11	21,744	—	230	—	230
Stock-based compensation	—	—	1,030	—	1,030
Net loss	—	—	—	(8,471)	(8,471)
Balance as of June 30, 2019	15,687,546	$ 16	$ 64,536	$ (39,395)	$ 25,157
Issuance of common shares, net of issuance costs of $1,991	2,347,479	2	17,503	—	17,505
Stock-based compensation	—	—	776	—	776
Net loss	—	—	—	(9,018)	(9,018)
Balance as of September 30, 2019	18,035,025	$ 18	$ 82,815	$ (48,413)	$ 34,420

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

	Nine months ended September 30,	
	2019	**2018**
CASH FLOWS FROM OPERATING ACTIVITIES:		
Net loss	$ (24,693)	$ (12,195)
Reconciliation of net loss to net cash used in operating activities		
Depreciation and amortization	218	9
Stock-based compensation expense	2,488	2,949

Changes in operating assets and liabilities:		
Prepaid expenses and other assets	(618)	(515)
Accounts payable, accrued expenses and other	3,249	584
Net cash used in operating activities	(19,356)	(9,168)
CASH FLOWS FROM INVESTING ACTIVITIES:		
Property and equipment, net	(868)	(182)
Net cash used in investing activities	(868)	(182)
CASH FLOWS FROM FINANCING ACTIVITIES:		
Proceeds from issuance of common stock, net	17,736	56,512
Exercise of options	1	—
Due to/from Parent	174	(556)
Note Payable — Parent	—	(371)
Net cash provided by financing activities	17,911	55,585
Net (decrease) increase in cash and cash equivalents	(2,313)	46,235
Cash and cash equivalents, beginning of the period	42,565	887
Cash and cash equivalents, end of the period	$ 40,252	$ 47,122
Supplemental cash flow information:		
Interest paid	$ 47	$ 1
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

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