August 14, 2020

BioXcel Therapeutics Reports Second Quarter 2020 Financial Results and Provides Business Update

SERENITY I & II Phase 3 trials of BXCL501 achieved all primary and secondary endpoints; New Drug Application (“*NDA”) submission to U.S. Food and Drug Administration (“FDA”) planned for Q1 2021*

Initiating the third dose cohort, 90 mcg, of the TRANQUILITY trial in elderly dementia patients

Initiated a separate efficacy cohort in the Phase 2 trial of BXCL701 in combination with pembrolizumab (“KEYTRUDA®”) *to include patients with castrate-resistant prostate cancer (“CRPC”)*

Strengthened balance sheet through follow-on offering raising approximately $200 million in gross proceeds

Company to host conference call today at 8:30 a.m. ET

NEW HAVEN, Conn., Aug. 14, 2020 (GLOBE NEWSWIRE) -- 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 second quarter ended June 30, 2020 and provided an update on key strategic and operational initiatives.

“Since inception, this is one of the most exciting times in the Company’s history,” stated Vimal Mehta, Chief Executive Officer of BTI. “First and foremost, we reported positive results from our two pivotal SERENITY trials last month that showed a robust treatment effect in reducing acute agitation in schizophrenia and bipolar disorder patients. We look forward to submitting an NDA for BXCL501 to the FDA in the first quarter of 2021 and, in preparation for a potential approval, we have made two key hires to lead commercialization and medical affairs. At the same time, we are advancing the TRANQUILITY and RELEASE trials in dementia and opioid withdrawal patients, respectively, and plan to initiate a trial in agitation associated with delirium patients this year, highlighting this candidate’s expected versatility across a wide range of diagnoses. We also continue to explore alternative indications associated with agitation, including alcohol withdrawal, post-traumatic stress disorder, traumatic brain injury and phobias. Finally, the successful completion of our recent follow-on equity offering puts us in a strong cash position to support BXCL501’s commercial launch, if approved, and our aggressive indication expansion strategy.”

Dr. Mehta continued, “In addition, our immuno-oncology program is advancing. The Phase 2 efficacy trial of BXCL701 and KEYTRUDA® for treatment emergent Neuroendocrine

Prostate Cancer continues to progress on track, and we have expanded this study to include a separate cohort of men with CRPC. Our MD Anderson-led Phase 2 basket trial in advanced solid tumors is also making great strides, now having met the efficacy bar in both arms necessary for the study to proceed to completion. We believe BXCL701 has the potential to bridge innate and adaptive immunity, as demonstrated by the increase in IL-18 from baseline, and we look forward to reporting initial efficacy data from both trials later this year."

Second Quarter 2020 and Recent Highlights

BXCL501-Neuroscience Program

BXCL501 is an investigational sublingual thin film of dexmedetomidine, a selective alpha-2 adrenergic receptor agonist, designed for the treatment of acute agitation. The Company believes BXCL501 may directly target a causal agitation mechanism.

- In July 2020, BTI reported that the pivotal Phase 3 SERENITY trials for the acute treatment of agitation in patients with schizophrenia and bipolar disorder met the primary, key secondary and exploratory endpoints. BXCL501 was well tolerated, with rapid and durable reductions in agitation. The Company has a pre-NDA meeting with the FDA this October and plans to submit an NDA for both indications in the first quarter of 2021.
- The Company is initiating a third dose cohort, 90 mcg, in the TRANQUILITY study, a Phase 1b/2 trial of BXCL501 for the acute treatment of agitation associated with dementia. The adaptive trial is designed to identify the most effective and tolerable dose in this elderly patient population. BTI has successfully completed two lower-dose cohorts (30 mcg and 60 mcg) in a total of 30 patients. Based on findings from the 90 mcg cohort, the Company expects to report topline results in the fourth quarter of 2020, or, if needed, proceed to an additional dose cohort.
- In June 2020, the first patient was enrolled in the RELEASE study, a Phase 1b/2 trial of BXCL501 for the acute treatment of opioid withdrawal symptoms, with the third cohort currently enrolling (90 mcg twice a day, 12 hours apart). The Company expects to report topline results from the study in the first quarter of 2021.
- The Company expects to initiate a Phase 2 trial of BXCL501 in patients with agitation associated with delirium later this year. The planned study population will include ICU patients with or without COVID-19. This potential indication may offer synergy with the commercial infrastructure being developed to support our first NDA.
- The Company received a Notice of Allowance from the U.S. Patent and Trademark office for patient application No. 16/453,679 related to BXCL501. The patent is expected to cover film formulations containing Dex and methods of treating agitation using such film formulations. The patent, which is anticipated to be issued in the third quarter of 2020, is expected to extend IP protection until 2039.

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 2 portion of the Phase 1b/2 trial of BXCL701 in combination with pembrolizumab (KEYTRUDA®) for treatment emergent Neuroendocrine Prostate Cancer (tNEPC) is advancing on track. Recently, BTI expanded the trial to include a separate cohort for CRPC (adenocarcinoma) patients who have failed taxane-based chemotherapy and up to two lines of second generation androgen pathway blockers, based on preliminary evidence of activity in this patient population in the safety cohort. The CRPC cohort is expected to run concurrently with the tNEPC cohort. Initial efficacy data from this trial are expected to be reported later this year.
- The open label Phase 2 basket trial evaluating the combination of BXCL701 and KEYTRUDA® in patients with advanced solid cancers is proceeding, with early signs of clinical activity in several difficult-to-treat cancers. This study, which is being conducted at the MD Anderson Cancer Center, consists of two arms: checkpoint naïve patients and patients who are refractory to checkpoint therapy. In June, the safety portion of the trial was completed and in August, the efficacy bar was met for both arms of the trial, allowing the study to proceed to completion. Initial efficacy data are expected to be presented at a scientific conference later this year.
- 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 second line pancreatic cancer is expected to begin following Nektar and Pfizer's Phase 1b safety trial of a double combination of bempegaldesleukin and avelumab, pending the outcome of that trial.

Corporate Highlights

- In July 2020, the Company raised gross proceeds of approximately $200 million in connection with its common stock offering. BTI believes that the proceeds from this offering, together with current reserves, provide cash runway well into 2022 to fund key clinical, regulatory, operational, and commercial activities.
- In June 2020, William Kane was appointed as Executive Vice President and Chief Commercial Officer of BTI. Mr. Kane brings over three decades of product commercialization experience in the pharmaceutical industry, with a proven track record in bringing neuropsychiatric drugs to market.
- In June 2020, Reina Benabou, M.D., Ph.D. was appointed as Senior Vice President and Chief Development Officer of BTI. Dr. Benabou has over 20 years of experience in directing drug development programs and implementing medical affairs strategies for product commercialization in neurology and psychiatry.

COVID-19

During the second quarter of 2020, the Company remained committed to ensuring the health and safety of its patients, investigators and employees. BTI continued to assess the impact of the COVID-19 pandemic to best mitigate risk, while continuing business operations. Beginning at the end of the second quarter, BTI began to slowly bring a limited number of staff back to the Company's office. This return to work is scheduled to be completed in September 2020. To date, the Company's business and operations has only been minimally impacted and have not experienced any significant delays to our ongoing or planned clinical trials, except for challenges in accessing elderly care facilities; however, this could rapidly change.

Second Quarter 2020 Financial Results

BTI reported a net loss of $21.4 million for the second quarter of 2020, compared to a net loss of $8.5 million for the same period in 2019. The second quarter 2020 results include approximately $2.0 million in non-cash stock-based compensation, compared to $1.0 million for the same period in 2019.

Research and development expenses were $17.9 million for the second quarter of 2020, compared to $6.5 million for the same period in 2019. The increase was primarily due to increases in clinical trial activity.

General and administrative expenses were $3.5 million for the second quarter of 2020, as compared to $2.1 million for the same period in 2019. The increase was primarily due to salaries, non-cash compensation costs and professional fees.

Total operating expenses for the second quarter of 2020 were approximately $21.4 million, compared to total operating expenses of approximately $8.6 million for the same period in 2019.

As of June 30, 2020, cash and cash equivalents totaled approximately $65.5 million. This does not include the $187.5 million in net proceeds generated from our equity offering that closed on July 31, 2020.

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 at least August 28, 2020.

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, applications and trials for BXCL501 and BXCL701, the Company’s cash runway, the impact of the COVID-19 pandemic on the Company’s business, financial results and financial

condition, the Company's future growth, corporate strategy and position to execute on key milestones and the Company's intellectual property portfolio. 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; undesirable side effects caused by BTI's product candidates; 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; impacts from the COVID-19 pandemic; 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 March 31, 2020 as such factors may be updated from time to time in its other filings with the SEC, including, but limited to, its Quarterly Report on Form 10-Q for the quarterly period ended June 30, 2020, which are accessible on the SEC's website at *www.sec.gov* and the Investors section of our website at *www.bioxceltherapeutics.com*.

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)

	June 30, 2020 (unaudited)	December 31, 2019
ASSETS		

Current assets		
Cash and cash equivalents	$ 65,495	$ 32,426
Prepaid expenses and other current assets	2,818	1,681
Total current assets	68,313	34,107
Property and equipment, net	997	1,041
Operating lease right-of-use asset	1,101	1,193
Other assets	51	51
Total assets	**$ 70,462**	**$ 36,392**
LIABILITIES AND STOCKHOLDERS' EQUITY		
Current liabilities		
Accounts payable	$ 10,434	$ 4,953
Accrued expenses	4,527	3,120
Due to Parent	72	64
Other current liabilities	1,128	331
Total current liabilities	16,161	8,468
Operating lease liability	907	1,029
Total liabilities	**17,068**	**9,497**
Stockholders' equity		
Preferred stock, $0.001 par value, 10,000,000 shares authorized; no shares issued or outstanding	—	—
Common stock, $0.001 par value, 50,000,000 shares authorized; 20,352,913 and 18,087,382 shares issued and outstanding as of June 30, 2020 and December 31, 2019, respectively	20	18
Additional paid-in-capital	146,392	83,565
Accumulated deficit	(93,018)	(56,688)
Total stockholders' equity	53,394	26,895
Total liabilities and stockholders' equity	**$ 70,462**	**$ 36,392**

BIOXCEL THERAPEUTICS, INC.

STATEMENTS OF OPERATIONS

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

	Three Months Ended June 30,		Six Months Ended June 30,	
	2020	2019	2020	2019
Revenues	$ —	$ —	$ —	$ —
Operating costs and expenses				
Research and development	17,906	6,506	30,277	12,180
General and administrative	3,529	2,129	6,154	3,874
Total operating expenses	21,435	8,635	36,431	16,054
Loss from operations	(21,435)	(8,635)	(36,431)	(16,054)
Other income				

Dividend and interest income, net	16	164	101	379
Net loss	$ (21,419)	$ (8,471)	$ (36,330)	$ (15,675)
Net loss per share attributable to common stockholders basic and diluted	$ (1.06)	$ (0.54)	$ (1.85)	$ (1.00)
Weighted average shares outstanding - basic and diluted	20,293,216	15,668,588	19,619,145	15,666,190

BIOXCEL THERAPEUTICS, INC.

STATEMENTS OF CHANGES IN STOCKHOLDERS' EQUITY

(amounts in thousands, except shares)

(unaudited)

	Common Stock		Additional Paid in	Accumulated	
	Shares	Amount	Capital	Deficit	Total
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
Balance as of December 31, 2019	18,087,382	$ 18	$ 83,565	$ (56,688)	$ 26,895
Issuance of common stock, net of issuance costs of $4,789	2,300,000	2	68,809		68,811
Purchase and cancellation of shares from BioXcel Corporation	(300,000)	—	(9,024)		(9,024)
Stock-based compensation	—	—	776	—	776
Exercise of stock options	95,000	—	39	—	39
Net loss	—	—	—	(14,911)	(14,911)
Balance as of March 31, 2020	20,182,382	$ 20	$ 144,165	$ (71,599)	$ 72,586
Stock-based compensation		—	1,956		1,956

Exercise of stock options	170,531	—	271		271
Net loss		—		(21,419)	(21,419)
Balance as of June 30, 2020	20,352,913	$ 20	$ 146,392	$ (93,018)	$ 53,394

BIOXCEL THERAPEUTICS, INC.

STATEMENTS OF CASH FLOWS

(amounts in thousands)

(unaudited)

	Six months ended June 30,	
	2020	**2019**
CASH FLOWS FROM OPERATING ACTIVITIES:		
Net loss	$ (36,330)	$ (15,675)
Reconciliation of net loss to net cash used in operating activities		
Depreciation and amortization	95	122
Stock-based compensation expense	2,732	1,712
Changes in operating assets and liabilities:		
Prepaid expenses and other assets	(1,137)	(1,009)
Accounts payable, accrued expenses and other	7,663	3,153
Net cash used in operating activities	(26,977)	(11,697)
CASH FLOWS FROM INVESTING ACTIVITIES:		
Purchases of equipment and leasehold improvements	(51)	(825)
Net cash used in investing activities	(51)	(825)
CASH FLOWS FROM FINANCING ACTIVITIES:		
Proceeds from issuance of common stock, net of issuance costs	68,811	230
Purchase and cancellation of shares from BioXcel Corporation	(9,024)	—
Deferred offering expense	—	(378)
Due to Parent	—	69
Exercise of options	310	1
Net cash provided by financing activities	60,097	(78)
Net (decrease) increase in cash and cash equivalents	33,069	(12,600)
Cash and cash equivalents, beginning of the period	32,426	42,565
Cash and cash equivalents, end of the period	$ 65,495	$ 29,965
Supplemental cash flow information:		
Interest paid	$ 18	$ 29

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