Eleven Biotherapeutics Reports Second Quarter 2017 Financial Results

August 14, 2017

-- AnnouncedData and Safety Monitoring Board Recommendation to Continue Phase 3 Registration Trial of Vicinium™ as Planned --

-- Entered Collaboration with National Cancer Institute and AstraZeneca to Evaluate Vicinium in Combination with Imfinzi™ (Durvalumab) in Patients with Non-Muscle Invasive Bladder Cancer --

-- Management to Host Conference Call Today at 8:00 a.m. ET --

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Aug. 14, 2017-- Eleven Biotherapeutics, Inc. (NASDAQ:EBIO), a late-stage clinical oncology company advancing a broad pipeline of novel product candidates based on its Targeted Protein Therapeutics (TPTs) platform, today reported financial results for the quarter ended June 30, 2017, and provided a corporate update.

“The second quarter was a very productive one for Eleven Bio. We significantly advanced our clinical development program for our lead candidate Vicinium™. Our independent Data Safety Monitoring Board (DSMB) had previously only reviewed safety data from our Phase 3 registration trial, but in June they undertook their first review of preliminary efficacy in conjunction with safety data, and recommended that the trial continue without modification. Patients with Bacillus Calmette-Guérin (BCG) unresponsive non-muscle invasive bladder cancer (NMIBC) have limited therapeutic options and frequently require cystectomies to prevent disease progression. Bladder removal, however, is a serious and life-altering surgery associated with significant morbidity and mortality. We consider the results of the DSMB’s review to be a very significant step, as they suggest that Vicinium may offer patients a positive non-surgical benefit/risk profile versus the standard of care,” said Stephen Hurly, President and Chief Executive Officer of Eleven Biotherapeutics.

“We were also very excited to announce that we entered into a collaboration with the National Cancer Institute (NCI) to evaluate Vicinium’s potential in combination with AstraZeneca’s immune checkpoint inhibitor, Imfinzi. One of the key differentiating attributes of our TPT platform is its dual mechanism of action. TPTs directly kill cancer cells via protein synthesis inhibition after targeted internalization, resulting in immunogenic cell death, which we believe sparks a second mechanism of anti-tumor activity via the patient’s own immune system. Together, our achievements this quarter represent important progress toward both our Vicinium monotherapy strategy and our combination strategy for our TPT platform more broadly. We look forward to announcing topline three-month complete response rate data from our Phase 3 registration trial next year.”

Second Quarter and Recent Business Highlights and Anticipated Upcoming Milestones:

Vicinium:

Vicinium is based on Eleven’s TPT technology. TPTs are fully biologic, single protein molecules that selectively bind to cell surface markers that are over-expressed on cancer cells. The TPTs are then internalized by cancer cells, and once inside, release highly cytotoxic payloads to selectively kill the cell while sparing non-targeted healthy cells. TPTs are specifically designed to improve upon and overcome the challenges of existing antibody drug conjugates (ADCs) by directly killing cancer cells, promoting systemic anti-tumor immune responses and delivering better tumor penetration with an improved payload that is capable of killing both dividing and non-dividing cancer cells. TPTs are designed to be stable by using a single protein structure that Eleven believes will improve safety and tolerability.

At the American Association for Cancer Research Annual Meeting in April 2017, Eleven presented new preclinical data demonstrating that cancer cells treated with VB4-845, the active pharmaceutical ingredient used to formulate Vicinium, induced the expression of HMGB1. HMGB1 is one of the three damage-associated molecular patterns (DAMPs) indicative of immunogenic cell death, which is recognized by immunologists to actively engage the host immune system and promote anti-tumor immune responses. This is especially meaningful because it builds on prior research in which Eleven observed the two other DAMPs markers – cell surface expression of calreticulin and extracellular release of ATP – following treatment with VB4-845. The induction of the three DAMPs that comprise the hallmark of immunogenic cell death suggests that TPTs are capable of inducing host anti-tumor immune responses, which can promote the function of immuno-oncology agents like checkpoint inhibitors. This supports the hypothesis that TPTs not only directly kill tumor cells, but also induce a host immune cell-mediated anti-tumor response. This suggests that they are differentiated from existing treatments, and that they may have synergy with checkpoint inhibitors and other immuno-oncology compounds.

Vicinium is a single protein TPT molecule composed of an antibody fragment genetically fused to a potent cytotoxic payload. Vicinium selectively binds to epithelial cell adhesion molecules (EpCAM), a cell surface marker that is highly expressed on many cancers, including high grade NMIBC, but is present at minimal to no levels on healthy bladder tissue. After binding to EpCAM on the surface of the tumor cell, Vicinium is internalized into the cell where its potent cytotoxic cell killing payload, Pseudomonas Exotoxin A (ETA), is released, disrupting protein synthesis and leading to cell death. Vicinium is currently in a Phase 3 registration trial for the treatment of high-grade NMIBC in subjects who have previously received a minimum of two courses of BCG and whose disease is now BCG-unresponsive.

- Complete enrollment for Phase 3 registration clinical trial expected in first quarter of 2018
- Topline three-month data from Phase 3 registration clinical trial expected mid-2018; topline 12-month data now expected in second quarter of 2019

In June 2017, Eleven announced that its ongoing Phase 3 registration trial of Vicinium exceeded 50% enrollment. Eleven also announced that the DSMB for the trial reviewed available data to assess the risk/benefit to patients on drug, and recommended that the trial continue without modification. The ongoing Phase 3 registration trial is a single-arm study evaluating Vicinium in patients with high-grade NMIBC, who have previously received a minimum of two courses of BCG and whose disease is now BCG-unresponsive. Eleven plans to enroll 134 patients at over 70 centers in the United States and Canada. The trial's primary endpoints are the complete response rate and duration of response in patients with carcinoma- *in-situ* (CIS). Secondary endpoints include time to disease recurrence and event-free survival.

Also in June 2017, Eleven announced the signing of a Cooperative Research and Development Agreement (CRADA) with the NCI for the development of Vicinium in combination with AstraZeneca's immune checkpoint inhibitor, Imfinzi, for the treatment of NMIBC. Under the terms of the CRADA, the NCI will conduct a Phase 1 clinical trial in patients with high-grade NMIBC to evaluate the safety, efficacy and biological correlates of Vicinium in combination with Imfinzi. Patients will be evaluated for safety and efficacy throughout the study. A broad biomarker program will provide information regarding Vicinium's ability to drive host anti-tumor immune responses. This will not only allow Eleven to assess the ability of Vicinium to work synergistically with Imfinzi, but will also help guide the identification of other immuno-oncology pathways and drugs that could be attractive candidates for combination studies with Vicinium and other TPTs. The decision to evaluate Vicinium in combination with Imfinzi is based on preclinical data, which suggest that Eleven's TPTs induce a host immune cell-mediated anti-tumor response, and thus may have synergy with checkpoint inhibitors and other immuno-oncology compounds.

TPT Pipeline:

Eleven's pipeline includes additional locally delivered product candidates, as well as a systemic platform **.** Given Eleven's enthusiasm for quickly driving the development of Vicinium forward, the Company is focusing its resources on the continued advancement of its Phase 3 registration trial at this time and temporarily holding the development of its earlier-stage product candidates, Proxinium and VB6-845d. The Company looks forward to moving these forward at the appropriate time.

Proxinium:

- Proxinium is a single protein anti-EpCAM antibody fragment fused with ETA for the treatment of late-stage, EpCAM-expressing, recurrent or metastatic squamous cell carcinoma of the head and neck (SCCHN).
    - Proxinium has received Orphan Drug Designation from the U.S. Food and Drug Administration (FDA) and the European Medicines Agency (EMA).
    - In prior Phase 1 and 2 clinical trials, Proxinium demonstrated anti-tumor activity both injected as well as un-injected tumors.
    - At the appropriate time, the Company plans to evaluate Proxinium in a Phase 1/2a clinical trial in combination with a checkpoint inhibitor.

VB6-845d:

- VB6-845d is a systemically-administered TPT utilizing a proprietary, highly potent, de-immunized plant toxin, deBouganin, for the treatment of solid tumors.
    - At the appropriate time, the Company plans to file an investigational new drug (IND) application for VB6-845d and initiate a Phase 1 trial.

Corporate:

- In May 2017, Eleven announced the appointment of David Brooks, M.D., Ph.D., as Senior Vice President, Clinical Development. Dr. Brooks is responsible for the execution of Eleven's ongoing and planned clinical trials.

Second Quarter 2017 Financial Results:

- **Cash Position:** Cash and cash equivalents were $15.8 million as of June 30, 2017, compared to $25.3 million as of December 31, 2016.
- **Revenue:** Eleven did not record any revenue for the three months ended June 30, 2017, compared to revenue of $0.3 million for the same period in 2016. This decrease was due to the termination of Eleven's collaboration agreement with Thrombogenics N.V. in June 2016.
- **R&D Expenses:** Research and development expenses were $2.9 million for the three months ended June 30, 2017, compared to $3.3 million for the same period in 2016. This decrease was primarily due to a reduction in EBI-031 development expenses. Effective August 2016, F. Hoffmann-La Roche Ltd and Hoffmann-La Roche Inc. (Roche) is responsible for developing EBI-031 pursuant to Eleven's license agreement with Roche. This decrease was partially offset by increases in Vicinium-related development expenses, which began in September 2016.
- **G&A Expenses:** General and administrative expenses were $2.2 million for the three months ended June 30, 2017, compared to $3.5 million for the same period in 2016. This decrease was primarily due to a reduction in professional fees.
- **Net Loss:** Net loss applicable to common stockholders was $7.3 million, or $0.30 per share, for the three months ended June 30, 2017, compared to net loss applicable to common stockholders of $6.5 million, or $0.33 per share, for the same period in 2016.
- **Financial Guidance:** Based on current operating plans, Eleven expects to have cash to fund research and development programs and operations into early 2018.

Upcoming Events and Presentations:

- David Brooks, MD, PhD, SVP, Clinical Development. “Development of Vicinium, an Intravesical Anti-EpCAM Toxin Fusion Protein, in Phase 3 for Non-Muscle Invasive Bladder Cancer.” 18th Future Directions in Urology Symposium (FDUS 18), August 20-23, 2017 in Colorado Springs, Colorado.
- Gregory P. Adams, PhD, CSO. Keynote Presentation: “Enabling Effective Immuno-Oncology.” CHI’s 5th Annual Immunomodulatory Therapeutic Antibodies for Cancer, August 28-29, 2017 in Boston, Massachusetts.

Conference Call Information:

Eleven Biotherapeutics’ management team will host a conference call and audio webcast today at 8:00 a.m. ET to discuss the second quarter 2017 financial results and provide a corporate update. To access the conference call, please dial (844) 831-3025 (domestic) or (315) 625-6887 (international) at least five minutes prior to the start time and refer to conference ID 63779857.

An audio webcast of the call will also be available on the Investors & Media section of the Company’s website, www.elevenbio.com. An archived webcast will be available on the Company’s website approximately two hours after the event and will be available for 30 days.

About Eleven Biotherapeutics:

Eleven Biotherapeutics, Inc. is a late-stage, clinical oncology company advancing a broad pipeline of novel product candidates based upon the Company’s targeted protein therapeutics (TPTs) platform. The Company’s TPTs incorporate a tumor-targeting antibody fragment and a protein cytotoxic payload into a single protein molecule in order to achieve focused tumor cell killing. The Company believes its TPT approach offers significant advantages in treating cancer over existing therapeutic options. The Company believes its TPTs provide effective tumor targeting with broader cancer cell-killing properties than are achievable with small molecule payloads that require tumor cell proliferation and face multi-drug resistant mechanisms. Additionally, the Company believes that its TPT’s cancer cell-killing properties promote an anti-tumor immune response that will potentially combine well with immune oncology drugs such as checkpoint inhibitors. For more information, please refer to the Company’s website at www.elevenbio.com.

Cautionary Note on Forward-Looking Statements:

Any statements in this press release about future expectations, plans and prospects for the Company, the Company’s strategy, future operations, and other statements containing the words “anticipate,” “believe,” “estimate,” “expect,” “intend,” “may,” “plan,” “predict,” “project,” “target,” “potential,” “will,” “would,” “could,” “should,” “continue,” and similar expressions, constitute forward-looking statements within the meaning of The Private Securities Litigation Reform Act of 1995. Actual results may differ materially from those indicated by such forward-looking statements as a result of various important factors, including: the occurrence of any event change or other circumstances that could give rise to the termination of the License Agreement (License Agreement) with F. Hoffmann-La Roche Ltd and Hoffman-La Roche Inc., the uncertainties inherent in receiving future payments pursuant to the License Agreement, the uncertainties inherent in the initiation and conduct of clinical trials, our ability to successfully develop our product candidates and complete our planned clinical programs, our ability to obtain marketing approvals for our product candidates, expectations regarding our ongoing clinical trials, availability and timing of data from clinical trials, whether interim results from a clinical trial will be predictive of the final results of the trial or results of early clinical studies will be indicative of the results of future studies, the adequacy of any clinical models, expectations regarding regulatory approvals, our ability to obtain, maintain and protect our intellectual property for our technology and products, availability of funding sufficient for the Company’s foreseeable and unforeseeable operating expenses and capital expenditure requirements, other matters that could affect the financial performance of the Company, other matters that could affect the availability or commercial potential of the Company’s product candidates and other factors discussed in the “Risk Factors” section of the Company’s Annual Report on Form 10-K, Quarterly Reports on Form 10-Q and other reports filed with the Securities and Exchange Commission. In addition, the forward-looking statements included in this press release represent the Company’s views as of the date hereof. The Company anticipates that subsequent events and developments will cause the Company’s views to change. However, while the Company may elect to update these forward-looking statements at some point in the future, the Company specifically disclaims any obligation to do so. These forward-looking statements should not be relied upon as representing the Company’s views as of any date subsequent to the date hereof.

ELEVEN BIOTHERAPEUTICS, INC.
CONDENSED CONSOLIDATED STATEMENTS OF OPERATIONS
(unaudited)
(in thousands, except per share data)

	Three Months Ended June 30,		Six Months Ended June 30,	
	2017	**2016**	**2017**	**2016**
Total revenue	$ -	$ 277	$ 425	$ 506
Operating expenses:				
Research and development	2,909	3,298	5,783	7,930
General and administrative	2,241	3,471	4,454	5,618
(Gain) loss from change in fair value of contingent consideration	2,200	-	3,700	-
Total operating expenses	7,350	6,769	13,937	13,548
Loss from operations	(7,350)	(6,492)	(13,512)	(13,042)
Other income (expense), net	34	1	135	(1,023)
Net loss	$ (7,316)	$ (6,491)	$ (13,377)	$ (14,065)

Net loss per share —basic and diluted	$ (0.30)	$ (0.33)	$ (0.54)	$ (0.71)
Weighted-average number of common shares used in net loss per share —basic and diluted	24,685	19,874	24,648	19,756

ELEVEN BIOTHERAPEUTICS, INC.
CONDENSED CONSOLIDATED BALANCE SHEETS
(unaudited)
(in thousands)

	June 30, 2017	December 31, 2016
Assets		
Current assets:		
Cash and cash equivalents	$ 15,751	$ 25,342
Prepaid expenses and other current assets	707	585
Total current assets	16,458	25,927
Property and equipment, net	632	796
Restricted cash	10	10
Intangible assets	60,500	60,500
Goodwill	17,371	16,864
Other assets	77	-
Total assets	$ 95,048	$ 104,097
Liabilities and stockholders' equity		
Current liabilities:		
Accounts payable	$ 1,245	$ 1,667
Accrued expenses	2,492	1,774
Deferred revenue	-	425
Due to related party	118	114
Total current liabilities	3,855	3,980
Other liabilities	148	-
Warrant liability	-	5
Deferred tax liability	16,335	16,335
Contingent consideration	48,800	45,100
Stockholders' equity:		
Common stock	25	25
Additional paid-in capital	162,564	161,963
Accumulated deficit	(136,679)	(123,311)
Total stockholders' equity	25,910	38,677
Total liabilities and stockholders' equity	$ 95,048	$ 104,097

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