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Eleven Biotherapeutics to Collaborate with AstraZeneca and the National Cancer Institute on Development of ViciniumTM in Combination with Durvalumab for the Treatment of Non-Muscle Invasive Bladder Cancer

-- Cooperative Research and Development Agreement to Evaluate Activity of Eleven's Targeted Therapeutic Vicinium in Combination with the Immune Checkpoint Inhibitor Durvalumab --

CAMBRIDGE, Mass.--(BUSINESS WIRE)-- 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 announced the signing of a Cooperative Research and Development Agreement (CRADA) with the National Cancer Institute (NCI) on the development of Eleven's targeted therapeutic, Vicinium™ in combination with AstraZeneca's immune checkpoint inhibitor, Imfinzi™ (durvalumab), for the treatment of non-muscle invasive bladder cancer (NMIBC).

"Despite current therapies and surgical regimens, there remains a large unmet need for patients with recurring or progressing NMIBC that is no longer responding to Bacillus Calmette-Guérin (BCG)," said Stephen Hurly, President and Chief Executive Officer of Eleven Biotherapeutics. "While we remain internally focused on advancing our Phase 3 registration trial of Vicinium as a monotherapy, preclinical data suggests that Vicinium also has the ability to potentiate the activity of immuno-oncology agents. We are pleased to enter into this collaboration with the National Cancer Institute and AstraZeneca, which broadens the scope of our ongoing clinical program and enables us to evaluate Vicinium together with Imfinzi, a PD-L1 checkpoint inhibitor. We look forward to generating additional data, as we continue to advance Vicinium and work expeditiously to bring it forward as a new treatment option for patients with NMIBC."

Vicinium, like Eleven's other TPTs, is a single protein 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 that 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.

At the American Association for Cancer Research Annual Meeting in April 2017, new preclinical data were presented demonstrating that cancer cells treated with VB4-845, the active pharmaceutical ingredient used to formulate Vicinium, undergo immunogenic cell death (ICD). ICD is known to stimulate host immune responses against cancer. This supports the hypothesis that Eleven's 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.

Under the terms of the CRADA, the NCI, led by principal investigator Dr. Piyush Agarwal of the NCI Center for Cancer Research, Urologic Oncology Branch, will conduct a Phase 1 clinical trial in patients with high-grade NMIBC to evaluate the safety, efficacy, and biological correlates of the Vicinium and durvalumab combination therapeutic strategy. Patients will be evaluated for response and recurrence throughout the study. For referrals, please contact Sonia Bellfield, Research Nurse, at 301-435-6255.

Vicinium is currently in a Phase 3 registration trial for the treatment of high-grade NMIBC. Eleven expects to complete patient enrollment in the second half of 2017, and to report topline 3-month data in the second quarter of 2018. Imfinzi, in development by AstraZeneca and its biologic research arm, MedImmune, is a human monoclonal antibody directed against programmed death ligand-1 (PD-L1), that has accelerated approval by the FDA for the treatment of patients with locally advanced or metastatic urothelial carcinoma who have disease progression during or following platinum-containing chemotherapy, or have disease progression within 12 months of neoadjuvant or adjuvant treatment with platinum-containing chemotherapy, regardless of PD-L1 status.

About Vicinium™

Vicinium is a single protein anti-epithelial cell adhesion molecule (anti-EpCAM) fusion protein fused with *Pseudomonas* Exotoxin A (ETA) designed to specifically target and deliver a potent anti-cancer payload directly into tumor cells. Vicinium is currently in a Phase 3 registration clinical trial for the treatment of high-grade non-muscle invasive bladder cancer (NMIBC) in subjects who have previously received two courses of Bacillus Calmette-Guérin (BCG) and whose disease is now BCG-

unresponsive. Eleven Biotherapeutics intends to enroll 134 subjects in the trial, including 77 subjects with carcinoma *in situ* (CIS), at over 70 centers in the United States and Canada. Primary and secondary endpoints include complete response (CR) rate in CIS subjects, time to disease recurrence and event free survival.

About Imfinzi™ (durvalumab)

Imfinzi (durvalumab, previously known as MEDI4736) is a human monoclonal antibody directed against PD-L1, which blocks the interaction of PD-L1 with PD-1 and CD80.

Durvalumab is also being tested in the 1st-line treatment of patients with unresectable and metastatic bladder cancer as a monotherapy and in combination with tremelimumab, a checkpoint inhibitor that targets CTLA-4, as part of the DANUBE Phase III trial, which had the last patient commenced dosing during the first quarter of 2017 (global trial, excluding China). Additional clinical trials are ongoing to investigate durvalumab as monotherapy or in combination in multiple solid tumours and blood cancers.

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 TPT platform. The Company's TPTs incorporate a tumor-targeting antibody fragment and a protein cytotoxic payload into a single protein molecule to achieve focused tumor cell killing. The Company believes its TPT approach offers significant advantages in treating cancer over existing ADC technologies. 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 resistance 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 immuno-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, 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.

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