Enrollment Completed in Phase 3 Registration Trial for Non-Muscle Invasive Bladder Cancer

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Eleven Biotherapeutics' VISTA Trial of Vicinium™ in NMIBC On-Track for Topline Three-Month Data in Mid-2018

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Mar. 12, 2018-- Eleven Biotherapeutics, Inc. (NASDAQ:EBIO), a late-stage clinical company advancing next-generation antibody-drug conjugate (ADC) therapies for the treatment of cancer, today announced that the company has completed enrollment in the VISTA Phase 3 registration trial of Vicinium™ in patients with non-muscle invasive bladder cancer (NMIBC) who have been previously treated with bacillus Calmette-Guérin (BCG). Bladder cancer is the sixth most common cancer in the United States and approximately 80 percent of bladder cancer patients are diagnosed with NMIBC. Vicinium is a fusion protein, designed to be a next-generation ADC, which specifically targets the epithelial cell adhesion molecule (EpCAM) antigens on the surface of bladder cancer cells to deliver a potent cytotoxin to those cells.

"Bladder cancer is one of the most prevalent cancers in the United States, yet there has been limited development of new therapeutic options for patients in more than 30 years," commented Donald Lamm, M.D., University of Arizona professor, director of BCG oncology and an investigator in the VISTA trial. "Today's standard-of-care for NMIBC provides initial responses in many patients; however, after BCG is no longer effective, there are no meaningful FDA-approved options except surgical removal of the bladder in high-risk patients. I am encouraged by the data demonstrated with Vicinium in prior trials and its potential to offer my patients an alternative to radical cystectomy."

"We believe Vicinium is the most advanced candidate in development for NMIBC and has the potential to be an effective and tolerable treatment for patients who have been previously treated with BCG," said Stephen Hurly, president and chief executive officer of Eleven Biotherapeutics. "The complete response rate and favorable safety seen in our Phase 2 trial were encouraging, and based on learnings from that trial, we modified the dosing regimen to potentially further enhance responses in the VISTA trial. With Phase 3 recruitment complete, we are on-track to report topline, three-month data in mid-2018, and look forward to further assessing Vicinium's potential in treating patients with this devastating cancer."

About the VISTA Clinical Trial

The VISTA trial is an open-label, multicenter, single-arm Phase 3 clinical trial evaluating the efficacy and tolerability of Vicinium™ in patients with high-risk non-muscle invasive bladder cancer (NMIBC) that is carcinoma in situ (CIS, cancer found on the inner lining of the bladder that has not spread into muscle or other tissue) or papillary (cancer that has grown from the bladder lining out into the bladder but has not spread into muscle or other tissue), who have been previously treated with bacillus Calmette-Guérin (BCG). The primary endpoint of the trial is the complete response rate in patients with CIS with or without papillary disease. Patients in the study receive locally administered Vicinium twice a week for six weeks, followed by once-weekly treatment for another six weeks, then treatment every other week for up to two years. Topline data assessing responses and durability of responses at three-months on treatment are expected in mid-2018, with 12-month data anticipated in mid-2019. For more information, please visit www.mybladdercancer.com.

About Vicinium™

Vicinium™, Eleven Biotherapeutics' lead product candidate, is a next-generation antibody-drug conjugate developed using the company's proprietary Targeted Protein Therapeutics platform. Vicinium is comprised of a recombinant fusion protein that targets epithelial cell adhesion molecule (EpCAM) antigens on the surface of tumor cells to deliver a potent protein payload, *Pseudomonas* Exotoxin A (ETA). Vicinium is constructed with a stable, genetically engineered linker to ensure the payload remains attached until it is internalized by the cancer cell, which is believed to decrease the risk of toxicity to healthy tissues, thereby improving its safety. In prior clinical studies conducted by Eleven Biotherapeutics, EpCAM has been shown to be overexpressed in non-muscle invasive bladder cancer (NMIBC) cells with minimal to no EpCAM expression observed on normal bladder cells. Eleven Biotherapeutics is currently conducting the Phase 3 VISTA trial, designed to support the registration of Vicinium for the treatment of NMIBC in patients who have previously received two courses of bacillus Calmette-Guérin (BCG) and whose disease is now BCG-unresponsive. Topline, three-month data from the trial are expected in mid-2018. The activity of Vicinium in BCG-unresponsive NMIBC is also being explored at the US National Cancer Institute in combination with AstraZeneca's immune checkpoint inhibitor durvalumab.

About Non-Muscle Invasive Bladder Cancer

Bladder cancer is the sixth most commonly diagnosed cancer in the United States, and approximately 80 percent of patients have non-muscle invasive bladder cancer (NMIBC). In NMIBC, cancer cells are in the lining of the bladder or have grown into the lumen of the bladder, but have not spread into muscle or other tissue. NMIBC primarily affects men and is associated with carcinogen exposure. Initial treatment includes surgical resection; however, there is a high rate of recurrence and more than 60 percent of all patients diagnosed with NMIBC will receive bacillus Calmette-Guérin (BCG) immunotherapy. While BCG is effective in many patients, challenges with tolerability have been observed and many patients will experience recurrence of disease. If BCG is not effective or a patient can longer receive BCG, the recommended option for treatment is radical cystectomy, the complete removal of the bladder.

About Eleven Biotherapeutics

Eleven Biotherapeutics, Inc. is a late-stage clinical company advancing next-generation antibody-drug conjugate therapies for the treatment of cancer based on the company's Targeted Protein Therapeutics platform. The company's lead program, Vicinium™, is currently in a Phase 3 registration trial for the treatment of non-muscle invasive bladder cancer, with topline data expected in mid-2018. Vicinium incorporates a tumor-targeting antibody

fragment and a protein cytotoxic payload into a single protein molecule designed to selectively, effectively and broadly kill cancer cells while sparing healthy cells. Additionally, Eleven Biotherapeutics believes that Vicinium's cancer cell-killing properties promote an anti-tumor immune response that may potentially combine well with immuno-oncology drugs, such as checkpoint inhibitors. For more information, please visit 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 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 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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