Press Release

Aeglea BioTherapeutics Doses Two Patients in Repeat Dose Part of Phase 1/2 Clinical Trial for the Treatment of Arginase 1 Deficiency

Topline data is expected in second half of 2018

AUSTIN, Texas, Sept. 06, 2017 (GLOBE NEWSWIRE) -- Aeglea BioTherapeutics, Inc., (NASDAQ:AGLE) a biotechnology company committed to developing enzyme-based therapeutics in the field of amino acid metabolism to treat rare genetic diseases and cancer, today announced that it has dosed two adults in the repeat dose part of its Phase 1/2 clinical trial of AEB1102 (pegzilarginase) for the treatment of patients with Arginase 1 Deficiency.

Initial results from two adult patients enrolled in Part 1 of the trial, who were administered single ascending doses, demonstrated that pegzilarginase was well tolerated and reduced arginine levels in the blood.

“The initiation of the repeat dose part of the study is important given the encouraging initial results seen with single doses of pegzilarginase, as it will provide important insights into the effects of longer term arginine reduction in patients with this serious and progressive disease,” said James Wooldridge, M.D., chief medical officer of Aeglea. “Additionally, we are continuing our discussions with the FDA about including pediatric patients in the trial, and have initiated activities in Europe and Canada to reach more patients with this very rare disease.”

“Although we have followed patients with Arginase 1 Deficiency for many years, we still have very little to offer these patients with a progressive neurological disease. We have therefore committed to help investigate pegzilarginase as a possible treatment option. Our patients are also excited about our working toward a treatment,” said Roberto Zori, M.D., professor and chief of the Division of Genetics and Metabolism at the University of Florida and an investigator in the trial.

About the Trial

The Phase 1/2, multicenter, single arm, open label, trial of AEB1102 will enroll at least 10 patients, adult and pediatric, with Arginase 1 Deficiency. The primary endpoint of the trial is safety and tolerability of intravenous administration of pegzilarginase in patients with Arginase 1 Deficiency. The trial will also evaluate the pharmacokinetic and pharmacodynamic effects of repeated doses of pegzilarginase including plasma arginine levels.

Please visit www.clinicaltrials.gov for more information.

About AEB1102 in Arginase 1 Deficiency

AEB1102 (pegzilarginase) is an engineered human arginase 1 enzyme designed to degrade the amino acid arginine. Aeglea is developing pegzilarginase to treat arginine excess in patients with Arginase 1 Deficiency, a urea cycle disorder caused by a mutation in the arginase 1 gene that leads to the inability to

degrade arginine. Pegzilarginase is intended for use as enzyme replacement therapy to restore the function of arginase 1 in patients by returning elevated blood arginine levels to the normal physiological range. Aeglea is currently recruiting patients for its ongoing Phase 1/2 trial for the treatment of Arginase 1 Deficiency. Data from the Phase 1 portion of the trial demonstrated that pegzilarginase has the ability to reduce blood arginine levels, providing initial human proof of mechanism.

About Aeglea BioTherapeutics

Aeglea is a biotechnology company committed to developing enzyme-based therapeutics in the field of amino acid metabolism to treat rare genetic diseases and cancer. The company's engineered human enzymes are designed to modulate the extremes of amino acid metabolism in the blood to reduce toxic levels of amino acids in inborn errors of metabolism or target tumor metabolism for cancer treatment. AEB1102, Aeglea's lead product candidate, is currently being studied in two ongoing Phase 1 clinical trials in patients with advanced solid tumors and acute myeloid leukemia/myelodysplastic syndrome (AML/MDS). Additionally, Aeglea is recruiting patients into its ongoing Phase 1/2 trial of AEB1102 for the treatment of patients with Arginase 1 Deficiency. The company is building a pipeline of additional product candidates targeting key amino acids, including AEB4104, which degrades homocystine, a target for an inborn error of metabolism, as well as two potential treatments for cancer, AEB3103, which degrades cysteine, and its oxidized form cystine, and AEB2109, which degrades methionine.

For more information, please visit http://aegleabio.com.

Safe Harbor / Forward Looking Statements

This press release contains "forward-looking" statements within the meaning of the safe harbor provisions of the U.S. Private Securities Litigation Reform Act of 1995. Forward-looking statements can be identified by words such as: "anticipate," "intend," "plan," "goal," "seek," "believe," "project," "estimate," "expect," "strategy," "future," "likely," "may," "should," "will" and similar references to future periods. These statements are subject to numerous risks and uncertainties that could cause actual results to differ materially from what we expect. Examples of forward-looking statements include, among others, statements we make regarding the timing and success of our clinical trials, and economic value of our lead product candidate or other product candidates. Further information on potential risk factors that could affect our business and its financial results are detailed in our most recent Quarterly Report on Form 10-Q for the quarter ended June 30, 2017 filed with the Securities and Exchange Commission (SEC), and other reports as filed with the SEC. We undertake no obligation to publicly update any forward-looking statement, whether written or oral, that may be made from time to time, whether as a result of new information, future developments or otherwise.

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