Press Release

View printer-friendly version

<< Back

Aeglea BioTherapeutics Doses First Pediatric Patient with Arginase 1 Deficiency in its Phase 1/2 Trial with Pegzilarginase (AEB1102)

Clinical Agreement Reached with FDA to Enable Pediatric Dosing

AUSTIN, Texas, Nov. 29, 2017 (GLOBE NEWSWIRE) -- Aeglea BioTherapeutics, Inc. (NASDAQ:AGLE), a biotechnology company committed to the discovery and development of engineered human enzyme therapeutics for patients with rare genetic diseases and cancer, today announced dosing of the first pediatric patient in its ongoing Phase 1/2 clinical trial in patients with Arginase 1 Deficiency and provided a regulatory update on its pegzilarginase (AEB1102) rare disease program.

Aeglea reached agreement with the FDA that available data supports the prospect for benefit in pediatric patients and amended its Phase 1/2 trial protocol to include pediatric dosing tiered by age beginning with patients aged 14 to 17, and continuing with patients aged 2 to 13.

“Dosing the first pediatric patient under the protocol amended during our work with the FDA is a critical step forward for our pegzilarginase program,” said Anthony Quinn, MB ChB, Ph.D., interim chief executive officer of Aeglea. “Arginase 1 Deficiency is a debilitating, progressive disease with significant morbidity and early mortality. This development allows us, for the first time, to begin generating data on the safety and activity of pegzilarginase in pediatric patients as we continue our mission to provide a treatment for patients living with this devastating disease. The team at Aeglea is excited and energized by this positive development, and we look forward to sharing updates from this trial, with topline results anticipated in the third quarter of 2018.”

“I am pleased that we can now include pediatric patients as we work to find an impactful treatment for this devastating disease,” said George Diaz, M.D., Ph.D. professor and chief, Division of Medical Genetics, Icahn School of Medicine at Mount Sinai. “Given that modest reductions in plasma arginine levels achieved with a highly restrictive diet appear to help some children clinically, I am excited, given the reductions in plasma arginine levels into the normal range achieved with single doses of pegzilarginase, to see the clinical impact of longer term dosing in these patients.”

About the Clinical Trial

The Phase 1/2, multicenter, single arm, open label trial of pegzilarginase will enroll approximately 10 patients, adult and pediatric, with Arginase 1 Deficiency in the United States, Canada, and Europe. The trial investigates both single ascending doses (Part 1) and repeated dosing (Part 2). 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 on plasma arginine levels, with topline results anticipated in the third quarter of 2018.

Please visit www.clinicaltrials.gov for more information.

About Pegzilarginase (AEB1102) in Arginase 1 Deficiency

Pegzilarginase (AEB1102) 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 results in 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 reduced 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. Pegzilarginase (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). An additional study of pegzilarginase combined with Merck’s pembrolizumab is expected to initiate in early 2018. Additionally, Aeglea is recruiting patients into its ongoing Phase 1/2 trial of pegzilarginase for the treatment of patients with Arginase 1 Deficiency. The company is building a pipeline of additional product candidates targeting key amino acids.

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, our ability to generate data on the safety and activity of pegzilarginase in pediatric patients, our ability to enroll patients into our clinical trials, and the potential therapeutic benefits 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 September 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.

Media Contact:
Kelly Boothe, Ph.D.
Pure Communications
415.946.1076
media@aegleabio.com

Investor Contact:
Charles N. York II
Chief Financial Officer
Aeglea BioTherapeutics
investors@aegleabio.com

Source: Aeglea BioTherapeutics, Inc.