

## Press Release

### Aeglea BioTherapeutics to Present Topline Data from Phase 1 Trial of AEB1102 for Treatment of Arginase I Deficiency at 2017 ACMG Annual Clinical Genetics Meeting

AUSTIN, Texas, March 23, 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 cancers, today announced topline data from a Phase 1 open-label study evaluating the safety and tolerability of its lead product candidate, AEB1102, for patients with Arginase I deficiency. The poster, entitled “Initial Results of a Phase I Open-Label Study of AEB1102 Enzyme Replacement Therapy in Adult Patients with Arginase I Deficiency” (Abstract #809), will be presented today at the 2017 American College of Medical Genetics and Genomics (ACMG) Annual Clinical Genetics Meeting being held in Phoenix, Arizona.

Arginase I deficiency is a rare genetic disorder characterized by heightened levels of arginine in the blood, which cause debilitating symptoms such as spasticity and neurocognitive deficits. AEB1102 is designed to address the root cause of the illness by lowering elevated blood arginine levels to the normal physiological range. The study showed that single, intermittent doses of AEB1102 were administered safely and tolerably, and resulted in the reduction of arginine in the blood to normal levels.

“Arginase I deficiency is a devastating disease that currently lacks adequate treatment options. We are pleased with the data from our Phase 1 study which show that not only was AEB1102 administered safely, but it also rapidly reduced blood arginine levels to the normal range in these patients,” said David G. Lowe, Ph.D., chief executive officer of Aeglea. “We are encouraged by the progress we have made with AEB1102 as we move one step closer to potentially providing these patients with a new and much needed treatment option.”

The study evaluated AEB1102 in two adult female patients who have moderate to severe neurocognitive and neurological deficits caused by Arginase I deficiency. Both patients adhere to a protein restriction and supplementation diet and use oral nitrogen scavengers.

The study allows patients to receive up to four intravenous doses of AEB1102 (0.015, 0.03, 0.06 and 0.1 mg/kg) with dose escalation at 2-week intervals. Dosing was stopped when plasma arginine levels decreased to less than 40 $\mu$ M. Patient A crossed the 40 $\mu$ M threshold after the third dose (0.06 mg/kg) and Patient B crossed the 40 $\mu$ M threshold after the second dose (0.03 mg/kg). At 168 hours after each dose, plasma arginine levels remained suppressed by 25 to 49 percent compared to pre-dose levels. The decrease in plasma arginine levels was proportional to the dose.

Treatment with AEB1102 was well tolerated with no related or possibly related adverse events or clinically significant abnormal laboratory results reported.

Additionally, Aeglea will present research data on newborn screening protocols for Arginase I deficiency in a poster presentation entitled “Newborn Screening for Arginase Deficiency in the U.S. – Where Do We Need to Go?” (Abstract #118) on Friday, March 24 at 10:30 a.m. MT at the 2017 ACMG Annual Clinical Genetics Meeting.

Both posters will be available on the [Investor Relations](#) section of the Aeglea BioTherapeutics website beginning on the day of their presentation at the 2017 ACMG Annual Clinical Genetics Meeting.

### **About AEB1102**

AEB1102 is an engineered human arginase I enzyme designed to degrade the amino acid arginine. Aeglea is developing AEB1102 to treat two extremes of arginine metabolism, including arginine excess in patients with Arginase I deficiency, as well as some cancers which have been shown to have a metabolic dependency on arginine. In patients with Arginase I deficiency, AEB1102 is intended for use as enzyme replacement therapy to restore the function of arginase I in patients and return 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 I deficiency. Aeglea is also conducting two Phase 1 trials in cancer patients with advanced solid tumors and with hematological malignancies to evaluate the safety and tolerability of AEB1102. Data from these trials demonstrated that AEB1102 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 I 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, the timing and success of our clinical trials, and the safety, potential therapeutic benefits and economic value of our product candidates. Further information on potential risk factors that could affect our business and its financial results are detailed in our most recent Annual Report on Form 10-K for the year ended December 31, 2016, 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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