

Aeglea BioTherapeutics Announces New Positive Interim Clinical Data and Improvements in Disease Manifestations for Ongoing Phase 1/2 Trial of Pegzilarginase in Patients with Arginase 1 Deficiency

Pegzilarginase is Effective in Sustainably Lowering Plasma Arginine Levels

Clinically Significant Improvements After Eight Doses Included Effects on Mobility and Adaptive Behavior

Company to Host Clinical Update Conference Call Today at 8:30 a.m. ET

AUSTIN, Texas, Oct. 17, 2018 (GLOBE NEWSWIRE) -- Aeglea BioTherapeutics, Inc.(NASDAQ: AGLE), a clinical-stage biotechnology company that designs and develops innovative human enzyme therapeutics for patients with rare genetic diseases and cancer, today announced new positive interim clinical data at the 2018 American Society of Human Genetics (ASHG) Conference from its ongoing Phase 1/2 trial of pegzilarginase, its lead investigational therapy, in patients with the rare genetic disease Arginase 1 Deficiency (ARG1-D).

“Our clinical experience with ARG1-D and pegzilarginase has advanced rapidly given the accelerated enrollment over the last few months. The developing efficacy profile is compelling, particularly given the short period of repeat dosing,” said Anthony G. Quinn, M.B Ch.B, Ph.D., president and chief executive officer of Aeglea. “We are pleased with the recently granted rare pediatric disease designation and we will continue to collaborate closely with the FDA and EMA on the design of our pivotal trial.”

“The arginine control we’ve seen with pegzilarginase in this study is remarkable – it’s well beyond what we have been able to achieve with the standard management of patients with Arginase 1 Deficiency,” stated Dr. Andreas Schulze, professor of pediatrics and biochemistry at the University of Toronto and Section Head, Metabolic Genetics at the Hospital for Sick Children in Toronto. “I’m truly encouraged by the improvements I have seen in one of our younger patients in terms of communication and walking, and I’m excited about moving the development of pegzilarginase into a pivotal study for patients with this rare and challenging disorder.”

Highlights of the ASHG presentation, entitled “Improvements in Arginase 1 Deficiency Related Disease Manifestations Following Plasma Arginine Reductions with Pegzilarginase: Early Phase 2 Results,” include the following:

- 100% of patients (6 out of 6) who completed Part 2 (repeat dose) of the study achieved consistent levels of reduced arginine, which was also accompanied by marked and sustained decreases in guanidino compounds (GCs);

- Comprehensive baseline profiling of patients with ARG1-D demonstrated quantifiable abnormalities in mobility and/or adaptive behavior in 94% of patients (15 out of 16);
- Pegzilarginase was well tolerated; most treatment-related AEs were mild, and hypersensitivity reactions were manageable with standard measures and all patients continued study treatment;
- 67% of patients (4 out of 6) had clinically meaningful improvements in mobility and/or adaptive behavior after only eight weeks of repeat dosing with pegzilarginase;
- Encouraging investigator and assessor feedback on a range of disease manifestation with improvements in alertness, communication, posture and walking.

Conference Call & Webcast Details

Aeglea will hold a clinical update conference call today, Wednesday, October 17, 2018 at 8:30 a.m. ET. To access the live conference call via phone, please dial 1-877-709-8155 (toll free) within the United States, or 1-201-689-8881 internationally. A replay of the call will be available through October 24, 2018 by dialing 1-877-660-6853 within the United States or 1-201-612-7415 internationally. The conference ID is 13683373.

To access the live and archived webcast of the presentation, please visit the [Presentations & Events](#) section of the Aeglea BioTherapeutics investor relations website. Please connect to the website at least 15 minutes prior to the presentation to allow for any software download that may be necessary.

About Pegzilarginase in Arginase 1 Deficiency

Pegzilarginase is an enhanced human arginase that enzymatically depletes the amino acid arginine. Aeglea is developing pegzilarginase for the treatment of patients with Arginase 1 Deficiency, a rare debilitating disease presenting in childhood with persistent hyperargininemia, severe progressive neurological abnormalities and early mortality. Pegzilarginase is intended for use as an enzyme replacement therapy in patients to reduce elevated blood arginine levels. Aeglea's interim Phase 1/2 data demonstrated clinical improvements and rapid and sustained lowering of plasma arginine in Arginase 1 Deficiency patients.

About Aeglea BioTherapeutics

Aeglea is a clinical-stage biotechnology company that designs and develops innovative human enzyme therapeutics for patients with rare genetic diseases and cancer. The Company is developing pegzilarginase, its lead investigational therapy, for the treatment of Arginase 1 Deficiency, as monotherapy in arginine-dependent cancers and in combination with an immune checkpoint inhibitor for small cell lung cancer. In addition, Aeglea has an active research pipeline of other human enzyme-based approaches in both therapeutic areas. 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. 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 potential therapeutic benefits and economic value of our lead product candidate or other product candidates and our eligibility to receive a priority review voucher. 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, 2018 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:

David Calusdian
Sharon Merrill Associates
617.542.5300
AGLE@investorrelations.com

Investor Contact:

Joey Perrone
Director, Finance & Investor Relations
Aeglea BioTherapeutics
investors@aegleabio.com



Source: Aeglea BioTherapeutics, Inc.