Aeglea BioTherapeutics Announces New Positive Phase 1/2 Data for Pegzilarginase in Patients with Arginase 1 Deficiency at 2019 Annual Meeting of the Society for Inherited Metabolic Disorders

Consistent, Marked, and Sustained Lowering of Elevated Plasma Arginine with Pegzilarginase

Clinically Impactful Responses Captured by Mobility and Adaptive Behavior Assessments

Management to Host Conference Call at 8:30 a.m. ET on Monday, April 8

AUSTIN, Texas, April 07, 2019 (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 presented new positive Phase 1/2 data for pegzilarginase in patients with Arginase 1 Deficiency (ARG1-D) at the 2019 Annual Meeting of the Society for Inherited Metabolic Disorders (SIMD) in Bellevue, Washington. The oral presentation was delivered by Dr. George Diaz, M.D., Ph.D., Division Chief of Medical Genetics in the Department of Genetics and Genomic Sciences at the Icahn School of Medicine at Mt. Sinai, New York, NY, and a Principal Investigator on the pegzilarginase Phase 1/2 trial.

The new Phase 1/2 data continues to demonstrate that pegzilarginase is highly effective in sustainably lowering plasma arginine, which is the primary endpoint in Aeglea's single, global pivotal Phase 3 PEACE trial. In addition, the new data shows that the marked improvement in plasma arginine control is accompanied by clinically meaningful responses in mobility and adaptive behavior, which are secondary endpoints in the PEACE trial. The treatment was generally well tolerated. Hypersensitivity reactions were infrequent, manageable with standard measures, and did not lead to treatment discontinuation.

"From the clinical perspective, the normalization I have seen in the arginine levels of my patients has been impressive," stated Dr. George Diaz, Division Chief of Medical Genetics in the Department of Genetics & Genomic Sciences at the Icahn School of Medicine at Mt. Sinai, New York, NY. "Additionally, the improvements in walking and social interactions, captured by assessments in mobility and adaptive behavior, provide evidence of clinical benefit and are important for patients and families."

"Aeglea's pegzilarginase is the first investigative therapy to address the markedly elevated levels of plasma arginine that are believed to be the key driver of clinical progression for patients with ARG1-D, a serious disease with significant complications that lead to early mortality," said Anthony G. Quinn, M.B. Ch.B, Ph.D., president and chief executive officer of Aeglea. "These highly encouraging Phase 1/2 results reaffirm our confidence in our pivotal Phase 3 PEACE trial design. We expect the data from this pivotal trial to support marketing applications for pegzilarginase in ARG1-D."

Highlights of the presentation, entitled "Sustained Reductions in Plasma Arginine Following Pegzilarginase Administration in Patients with Arginase-1 Deficiency are Accompanied by Improvements in Mobility and Adaptive Behavior," include the following:

- Plasma arginine reduction was statistically significant ($p<0.001$) at eight weeks with sustained control through longer term dosing.

- Pegzilarginase was generally well tolerated. Serious adverse events included hypersensitivity and hyperammonemia. Hypersensitivity reactions were infrequent, managed with standard treatment and did not lead to any patient discontinuations.
- Clinical responses were effectively captured using mobility and adaptive behavior assessments
  - Five of five (100%) and five of 14 (36%) patients showed mobility improvements at 20 weeks and 8 weeks, respectively.
  - One of five (20%) and three of ten (30%) patients showed adaptive behavior improvements at 20 weeks and 8 weeks, respectively.
  - Five of five (100%) and eight of 14 (57%) patients showed overall clinical response (mobility or adaptive behavior) at 20 weeks and 8 weeks, respectively.

Aeglea expects to begin the single, global pivotal Phase 3 PEACE trial in ARG1-D in the second quarter of 2019.

Clinical Update Conference Call & Webcast Details

Aeglea will hold a conference call on Monday, April 8, 2019 at 8:30 a.m. ET. To access the live conference call via phone, please dial (877) 709-8155 (toll free) within the United States, or (201) 689-8881 internationally. A replay of the call will be available through April 15, 2019 by dialing (877) 660-6853 within the United States or (201) 612-7415 internationally. The conference ID is 13689004.

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 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. Aeglea 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.

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 latest Phase 1/2 data demonstrated clinical improvements and rapid and sustained lowering of plasma arginine in Arginase 1 Deficiency patients. The Company intends to initiate its single, global pivotal Phase 3 PEACE trial designed to assess the effects of treatment with pegzilarginase versus placebo over 24 weeks with a primary endpoint of plasma arginine reduction.

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 related data, the timing of announcements and updates relating to our clinical

trials and related data, 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 Annual Report on Form 10-K for the year ended December 31, 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.

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