Press Release

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Aeglea BioTherapeutics Announces Positive 20-Dose Data for Pegzilarginase in Patients with Arginase 1 Deficiency at the 2019 SSIEM Symposium

Sustained Control of Plasma Arginine Accompanied by Clinical Response; Overall Clinical Responder Rate of 79%

Management to Host Conference Call at 9:00 a.m. ET on Tuesday, September 3

AUSTIN, Texas, Sept. 03, 2019 (GLOBE NEWSWIRE) -- Aeglea BioTherapeutics, Inc. (NASDAQ:AGLE), a clinical-stage biotechnology company that engineers next-generation human enzymes to provide solutions for diseases with unmet medical need, today presented positive 20-dose data on 14 patients from the Company's completed Phase 1/2 trial and ongoing Phase 2 open-label extension (OLE) trial for pegzilarginase in patients with Arginase 1 Deficiency (ARG1-D) at the 2019 Symposium of the Society for the Study of Inborn Errors of Metabolism (SSIEM).

"The arginine control I have seen in my patients continues to be 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. "I'm very encouraged by the improvements in mobility we observed in these patients after 20 doses. I believe the Phase 3 PEACE trial is well designed to capture these changes."

"We continue to see a transformative impact of pegzilarginase on plasma arginine control with accompanying improvements in important disease manifestations," said Anthony G. Quinn, M.B. Ch.B., Ph.D., president and chief executive officer of Aeglea. "The effectiveness of plasma arginine control and the 79% overall clinical response rate that we demonstrated in this trial gives us high confidence in the primary and secondary endpoints in our global pivotal Phase 3 PEACE trial."

Highlights of the poster presentation, entitled "Sustained Lowering of High Plasma Arginine Levels in ARG1-D with

Pegzilarginase Is Accompanied by Improvements in Disease Manifestations," include:

- Data from all patients following 20 doses of pegzilarginase continued to demonstrate marked and sustained reductions in plasma arginine
- 79% (11 of 14) of patients were clinical responders, using mobility assessment components that correspond with the pivotal PEACE trial secondary endpoint
- Pegzilarginase was well tolerated and the rates of treatment-related adverse events decreased over time. Serious adverse events included hypersensitivity and hyperammonemia, which were infrequent, managed with standard treatment and did not lead to any patient discontinuations.

We believe the improvements in arginine control and evidence of clinical benefit following pegzilarginase treatment provide further validation of the key endpoints and design elements of the pivotal Phase 3 PEACE trial and that the Phase 1/2 and OLE trials demonstrate the value of utilizing only three mobility assessment tools (6MWT, GMFM-D, or GMFM-E) to capture the clinical benefit of pegzilarginase.

Additionally, Aeglea announced that 10 patients have been dosed subcutaneously (sc) with pegzilarginase in the ongoing OLE trial in patients with ARG1-D. Highlights include:

- Subcutaneous administration of pegzilarginase controls plasma arginine similarly to IV administration, providing potential additional advantages such as improving compliance and/or convenience
- Pegzilarginase (sc) was well tolerated, with only four mild injection site reactions related to pegzilarginase in more than 200 injections to date
- All 10 eligible patients switched to, and remain on, pegzilarginase (sc), with no patient discontinuations

In June, Aeglea announced the dosing of the first patient in its pivotal PEACE trial, which is intended to further evaluate the efficacy and safety of pegzilarginase. The Company expects to continue discussions with the FDA on the pegzilarginase program and our next steps in the fourth quarter of 2019 or first quarter of 2020. The Company also expects to report topline data from the pivotal PEACE trial in the first quarter of 2021.

Conference Call & Webcast

Aeglea will hold a conference call on Tuesday, September 3, 2019 at 9:00 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 September 10, 2019 by dialing (877) 660-6853 within the United States or (201) 612-7415 internationally. The conference ID is 13694042.

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 of arginine metabolism 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 Phase 1/2 and Phase 2 open-label extension data evaluating pegzilarginase in patients with Arginase 1 Deficiency demonstrated clinical improvements and sustained lowering of plasma arginine. Aeglea is currently recruiting patients for 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.

About Aeglea BioTherapeutics

Aeglea is a clinical-stage biotechnology company that engineers next-generation human enzymes with enhanced properties and novel activity to provide solutions for diseases with unmet medical need. Aeglea is developing pegzilarginase, its lead product candidate, for the treatment of Arginase 1 Deficiency which has received both Rare Pediatric Disease and Breakthrough Therapy Designation. Aeglea has two programs in IND-enabling studies for Homocystinuria and Cystinuria and an active discovery pipeline. 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 our cash forecasts, the timing and success of our clinical trials and related data, the timing and expectations for regulatory submissions and approvals, timing and results of meetings with regulators, the potential for expeditated development and review of pegzilarginase as of a result of the Breakthrough Therapy designation, the timing of announcements and updates relating to our clinical trials and related data, our ability to enroll patients into our clinical trials, success in our collaborations 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 June 30, 2019 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
Senior Director, Finance & Investor Relations
Aeglea BioTherapeutics
investors@aegleabio.com

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