

Press Release

 [View printer-friendly version](#)

[<< Back](#)

Aeglea BioTherapeutics Announces 1-Year Data for Pegzilarginase in Patients with Arginase 1 Deficiency at the 6th Congress of the European Academy of Neurology

Pegzilarginase Showed Durable Clinical Response at 56 Week Analysis

All Patients Demonstrated a Marked and Sustained Reduction in Plasma Arginine

Favorable Safety Profile, Consistent with Previously Reported Results

AUSTIN, Texas, May 26, 2020 (GLOBE NEWSWIRE) -- Aeglea BioTherapeutics, Inc. (NASDAQ:AGLE), a clinical-stage biotechnology company developing a new generation of human enzyme therapeutics as innovative solutions for rare and other high-burden diseases, today announced a new 56 week analysis on Arginase 1 Deficiency (ARG1-D) patients who have been treated with pegzilarginase from the Company's completed Phase 1/2 clinical trial and the ongoing Phase 2 open-label extension study. The data were shared yesterday in a virtual, late-breaking oral presentation at the 6th Congress of the European Academy of Neurology.

"Arginase 1 Deficiency is a devastating disease that is frequently under diagnosed or misdiagnosed as more common neurological conditions, such as cerebral palsy, due to lack of awareness of this rare condition," said George Diaz, M.D., Ph.D., division chief of medical genetics in the Division of Medical Genetics and Genomics and Department of Genetics and Genomic Sciences at the Icahn School of Medicine at Mount Sinai, New York, NY. "Because of the condition's progressive nature, it is essential that patients be diagnosed early, and there is an urgent need for a therapy that addresses the underlying cause of the disease and improves clinical manifestations."

"The results of this long-term data demonstrate that treatment with pegzilarginase resulted in a durable clinical response, which is a critical factor in effectively treating a life-long, progressive condition," said Ravi M. Rao, M.B Ch.B PhD, chief medical officer of Aeglea. "We are also pleased to see that the lowering of arginine levels observed in the 20

week analysis were maintained through the 56 week analysis. These results align with the primary endpoint of PEACE, our ongoing pivotal Phase 3 clinical trial, and together with the durable clinical response bolsters our belief that pegzilarginase has the potential to be an impactful treatment for people living with Arginase 1 Deficiency.”

The presentation, titled “1 Year Data from First in Human Study of Pegzilarginase for the Treatment of Arginase 1 Deficiency (ARG1-D),” includes data on 13 patients treated with pegzilarginase who completed the 56 week treatment period (8 weeks Part 2 repeat dosing + 48 weeks open-label extension).

Highlights from the 56 week analysis include:

- A statistically significant reduction in plasma arginine from baseline was observed with a single dose, with continued improvement through the 20 and 56 week analyses
 - All 13 patients achieved plasma arginine levels within the target range (<200µM)
 - The median plasma arginine level was 99µM (normal range: 40-115µM)
- Eleven patients overall were clinical Responders (85%)
 - Mobility improvements were evaluated using three assessments: 6MWT (6 Minute Walk Test), GMFM (Gross Motor Function) Part D (standing) and Part E (walking, running, and jumping)
 - Mean change in 6MWT showed progressive improvement from baseline through 8, 20 and 56 week analyses
 - All six patients with significant mobility impairment at baseline (GMFCS [Gross Motor Function Classification System] Levels II and III) improved in the GMFM Part E; four out of six patients also improved in the GMFM Part D
- Pegzilarginase was shown to have a favorable safety profile with more than 750 doses administered
 - Safety profile is consistent with previously reported data
 - The most common treatment-related serious adverse events were hypersensitivity and hyperammonemia, both of which were infrequent, expected and managed with standard care; no treatment-related serious adverse events led to patient discontinuation
 - Most treatment-related adverse events were mild and decreased in frequency over time

The presentation is available for download on the [Presentations & Events](#) section of the Company’s website.

About the Phase 1/2 and Open-Label Extension Trial

The Phase 1/2, multicenter, single arm, open-label extension study of pegzilarginase enrolled patients aged 2 years and older with Arginase 1 Deficiency in the United States, Canada, and Europe. The trial investigates single ascending doses (Part 1), repeated weekly dosing for eight weeks (Part 2). The trial enrolled 16 adult and pediatric patients and 14 patients rolled over to the open-label extension. The primary endpoint of the trial is safety and tolerability of intravenous administration of pegzilarginase in patients with Arginase 1 Deficiency. The trial also evaluated the pharmacokinetic and pharmacodynamic effects of repeated doses of pegzilarginase on plasma arginine levels, and evaluation of clinical outcomes using several mobility assessments.

Please visit www.clinicaltrials.gov for more information.

About Pegzilarginase in Arginase 1 Deficiency

Pegzilarginase is an enhanced human arginase that enzymatically lowers levels of the amino acid arginine. Aeglea is developing pegzilarginase for the treatment of patients with Arginase 1 Deficiency (ARG1-D), 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 therapy to reduce elevated blood arginine levels in patients with ARG1-D. Aeglea’s Phase 1/2 and Phase 2 open-label extension data for pegzilarginase in patients with ARG1-D demonstrated clinical improvements and sustained lowering of plasma arginine. The Company’s single global pivotal

demonstrated clinical improvements and sustained lowering of plasma arginine. The Company's single, global pivotal Phase 3 PEACE trial is 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 BioTherapeutics is a clinical-stage biotechnology company redefining the potential of human enzyme therapeutics to benefit people with rare and other high burden diseases. Aeglea's lead product candidate, pegzilarginase, is in a pivotal Phase 3 trial for the treatment of Arginase 1 Deficiency and has received both Rare Pediatric Disease and Breakthrough Therapy Designation. The Company received approval of its Clinical Trial Application (CTA) for ACN00177 for the treatment of Homocystinuria by the United Kingdom's Medicines and Healthcare Products Regulatory Agency (MHRA). Aeglea has an active discovery platform, with the most advanced program for Cystinuria. 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 timing of announcements and updates relating to our clinical trials and related data, our ability to enroll patients into our clinical trials, the expected impact of the COVID-19 pandemic on our operations and clinical trials, success in our collaborations, the potential addressable markets of the our product candidates 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 March 31, 2020 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.
Director, Corporate Communications
Aeglea BioTherapeutics
512.399.5458
media@aegleabio.com

Investor Contact:

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



