Press Release

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Aeglea BioTherapeutics Receives FDA Breakthrough Therapy Designation for Pegzilarginase for Treatment of Arginase 1 Deficiency

Designation Follows Recently Reported ARG1-D Phase 1/2 Data Demonstrating Clinical Response in Patients Treated with Pegzilarginase

AUSTIN, Texas, July 24, 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 announced that the U.S. Food and Drug Administration (FDA) has granted Breakthrough Therapy Designation (BTD) to the Company's lead investigational therapy, pegzilarginase, for the treatment of Arginase 1 Deficiency (ARG1-D), a rare progressive disease presenting in early childhood that results in severe complications and early mortality.

"The emerging clinical data demonstrating favorable effects of pegzilarginase treatment on both plasma arginine levels and important disease manifestations has led to the FDA's designation of pegzilarginase as a breakthrough therapy," said Anthony G. Quinn, M.B. Ch.B, Ph.D., president and chief executive officer of Aeglea. "Breakthrough Therapy Designation provides a path to work closely with the FDA as we quickly advance this potentially transformative therapy for patients suffering from ARG1-D."

Breakthrough Therapy Designation is granted by the FDA to expedite the development and review of new therapies to treat serious or life-threatening conditions. The criteria for BTD require preliminary clinical evidence that demonstrates the therapy may have substantial improvement on at least one clinically significant endpoint over available therapy. For pegzilarginase, BTD was based on data from the Phase 1/2 and ongoing Phase 2 open-label extension clinical trials in patients with ARG1-D. This designation conveys all fast-track program features, as well as more intensive FDA guidance on an efficient drug development program, and eligibility for rolling review and priority review. Aeglea expects to continue discussions with the FDA on the pegzilarginase program and our next steps in the fourth quarter of 2019.

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 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 from baseline.

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 investigational therapy, for the treatment of Arginase 1 Deficiency, which has received both rare pediatric disease and breakthrough therapy designation. Aeglea has two pipeline programs in IND-enabling studies for Homocystinuria and Cystinuria and an active discovery pipeline. Pegzilarginase is also under investigation in cancer as combination therapy for small cell lung cancer. 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 March 31, 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.

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