FDA Grants Rare Pediatric Disease Designation to Pegzilarginase for Arginase 1 Deficiency

Aeglea Eligible to Receive a Priority Review Voucher

Company Plans to Initiate Pivotal Trial in First Half of 2019

AUSTIN, Texas, Oct. 01, 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 the U.S. Food and Drug Administration (FDA) has granted rare pediatric disease designation to the Company's lead product candidate, pegzilarginase, for the treatment of Arginase 1 Deficiency. This designation by the FDA confirms Aeglea's eligibility to receive a rare pediatric disease priority review voucher (PRV) upon approval of a biologics license application (BLA) for pegzilarginase. Arginase 1 Deficiency is a rare debilitating disease presenting in childhood with persistent hyperargininemia, severe progressive neurological abnormalities and early mortality.

"This designation underscores the seriousness of Arginase 1 Deficiency and its devastating impact on patients and families living with this disease," said Anthony G. Quinn, M.B Ch.B, Ph.D., president and chief executive officer of Aeglea. "We look forward to continued collaboration with the FDA as we advance pegzilarginase into a pivotal study in the first half of 2019."

Aeglea recently announced positive interim clinical data for its Phase 1/2 clinical trial of pegzilarginase in Arginase 1 Deficiency. In addition, the Company announced that it had fully enrolled its Phase 1/2 clinical trial of pegzilarginase by dosing its fifteenth patient, which exceeded the target enrollment of 10 patients. The Company plans to present additional interim clinical data for the Phase 1/2 trial at the 2018 American Society of Human Genetics (ASHG) Conference in October.

About Rare Pediatric Disease Designation

The FDA grants rare pediatric disease designation for diseases with serious or life-threatening manifestations that primarily affect fewer than 200,000 people in the United States and who are less than 18 years old. Under the FDA's Rare Pediatric Disease Priority Review Voucher program, a sponsor who receives an approval of a new drug application or biologics license application for a product for the prevention or treatment of a rare pediatric disease may be eligible for a voucher, which can be redeemed to obtain priority review for any subsequent marketing application or may be sold or transferred.

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. The Company's interim Phase 1/2 data demonstrated clinical improvements and rapid and sustained lowering of plasma arginine in Arginase 1 Deficiency patients.

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.

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