

# Aeglea BioTherapeutics Announces Approval of Clinical Trial Application for its Novel Engineered Human Enzyme Designed to Treat Homocystinuria (ACN00177)

---

AUSTIN, Texas, April 08, 2020 (GLOBE NEWSWIRE) -- Aeglea BioTherapeutics, Inc. (NASDAQ:AGLE), a clinical-stage biotechnology company developing next-generation human enzyme therapeutics as solutions for diseases with high unmet medical need, today announced the approval of its Clinical Trial Application (CTA) by the United Kingdom's Medicines and Healthcare Products Regulatory Agency (MHRA) for ACN00177, a novel engineered human enzyme therapy designed to treat Homocystinuria, a serious metabolic disorder characterized by elevated plasma homocysteine levels, leading to a wide range of life-altering complications and reduced life expectancy.

"The approval of the CTA for ACN00177 is an important step forward for our Homocystinuria program and for patients who are in need of new treatment options. Given these unprecedented times, our priorities are to minimize the risk of trial participants being exposed to COVID-19 and avoid further overburdening hospital staff," said Anthony G. Quinn, M.B. Ch.B., Ph.D., Aeglea's president and chief executive officer. "We are closely monitoring the situation with COVID-19. We remain committed to the patients we serve and are continuing our patient identification and administrative activities in support of this trial to ensure we are prepared to dose patients once circumstances permit."

There are currently limited treatment options for Homocystinuria. Disease management strategies – dietary protein restriction with amino acid replacement either alone or with vitamin B6, and betaine supplementation – are challenging, have poor adherence and many patients are unable to achieve target levels of homocysteine. There is an urgent need for new treatment options for patients in whom homocysteine levels remain high.

## **About ACN00177 in Homocystinuria**

Aeglea is developing ACN00177 for the treatment of patients with cystathionine beta synthase (CBS) deficiency, also known as Classical Homocystinuria. Homocysteine accumulation plays a key role in multiple progressive and serious disease-related complications, including thromboembolic vascular events, skeletal abnormalities (including severe osteoporosis), developmental delay, intellectual disability, lens dislocation and severe myopia. ACN00177 has been designed as a novel recombinant human enzyme, which degrades the amino acid homocysteine and its related homocystine dimer. With this mechanism, ACN00177 is intended to lower the abnormally high blood levels of homocysteine in patients with Homocystinuria. Preclinical data demonstrated that ACN00177 improved important disease-related abnormalities and survival in a mouse model of Homocystinuria. In April 2020, Aeglea announced that the United Kingdom's Medicines and Healthcare Products Regulatory Agency (MHRA) approved the Company's Clinical Trial Application.

## **About Aeglea BioTherapeutics**

Aeglea BioTherapeutics is a clinical-stage biotechnology company redefining the potential of human enzyme therapeutics to address rare and other high burden diseases with unmet medical need. Aeglea's lead product candidate, pegzilarginase, is in a Phase 3 pivotal trial for the treatment of Arginase 1 Deficiency and has received both Rare Pediatric Disease and Breakthrough Therapy Designation. Aeglea has an active discovery

platform with programs for Homocystinuria and Cystinuria. The Company's Clinical Trial Application (CTA) for ACN00177 for Homocystinuria has been approved by the United Kingdom's Medicines and Healthcare Products Regulatory Agency (MHRA). 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 expected impact of the COVID-19 pandemic on our operations and timing of our clinical trials, 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, success in our collaborations, the potential addressable markets of the Company's 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 Annual Report on Form 10-K for the year ended **December 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.

#### **Media Contact:**

Kelly Boothe, Ph.D.

Director, Corporate Communications

Aeglea BioTherapeutics

512.399.5458

[media@aegleabio.com](mailto:media@aegleabio.com)

#### **Investor Contact:**

Joey Perrone

Senior Director, Finance & Investor Relations

Aeglea BioTherapeutics

[investors@aegleabio.com](mailto:investors@aegleabio.com)



Source: Aeglea BioTherapeutics, Inc.

---