

# Press Release

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## Aeglea BioTherapeutics Presents New Phase 1/2 Trial Data Demonstrating Clinically Relevant Treatment Effects in Arginase 1 Deficiency Patients at the 2018 ACMG Annual Clinical Genetics Meeting

Clinical Outcome Assessment Tools Detected and Quantified Baseline Abnormalities

Clinically Relevant Improvement Seen in Multiple Neuromotor Tests

Company to Host Clinical Update Conference Call Today at 8:30 a.m. ET

AUSTIN, Texas, April 12, 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 is presenting new preliminary data that confirms the utility of standardized assessment tools in quantifying disease manifestations and that demonstrates clinically relevant treatment effects with pegzilarginase in two Arginase 1 Deficiency patients after only eight weeks of dosing. The Company is presenting the new data at the 2018 Annual Clinical Genetics Meeting of the American College of Medical Genetics and Genomics (ACMG) in Charlotte, North Carolina and will conduct a clinical update conference call today at 8:30 a.m. ET.

“We are building on our momentum from quarter one with these new clinical insights on the effects of pegzilarginase in Arginase 1 Deficiency patients,” said Anthony Quinn, M.B Ch.B, Ph.D., interim chief executive officer of Aeglea. “This new data links arginine reduction to meaningful clinical effects in the first two patients, and we look forward to advancing this key trial and our understanding of the clinical potential of pegzilarginase.”

### **Data Summary**

Baseline data in five patients indicated that clinical abnormalities in Arginase 1 Deficiency patients can be detected

and quantified using standardized assessment tools. Assessment tools used in the trial include:

- Six-Minute Walk Test (6MWT) was below age and gender match norms for all five patients
- Berg Balance Scale demonstrated impaired balance in two patients
- Gross Motor Function Measure (GMFM) total and Part E subscale (walking, running, and jumping) was abnormal in four of the five patients
- Purdue Pegboard test demonstrated fine motor ability was also quantifiably impaired in all five patients
- All five patients had markedly elevated plasma arginine and plasma guanidino compounds (GC)
- All patients had evidence of growth impairment with height in the lowest 10% for age and gender and protein intakes less than the prescribed restricted amounts, which we believe likely reflects an aversion to protein caused by the disease
- One patient had abnormal baseline ammonia and hepatic transaminases, which are also potentially important disease related biochemical manifestations
- Tests of neurocognition were abnormal in all subjects indicating significant cognitive impairment

Data are available for the first two patients and demonstrated clinically relevant treatment effects using standardized assessment tools:

- 6MWT demonstrated that two patients observed improvements on pegzilarginase. Patient 1 experienced a 31.4% improvement, from 102 to 134 meters, and Patient 2 experienced a 23.4% improvement, from 261 to 322 meters. Both observed improvements were well above the Minimal Clinically Important Difference (MCID) of 9% at eight weeks, with continued improvement, described above, measured at twenty weeks.
- Berg Balance Scale measured a clinically meaningful improvement in balance in Patient 1 who transitioned from a high risk to a medium risk of fall category. Patient 2 had a normal assessment which precluded demonstration of any improvement.
- The GMFM – Part E demonstrated clinically important improvement after the initial eight repeat doses with further improvement by twenty weeks in Patient 1. Patient 2 was already at the upper end of the scale and, as expected, no significant change was observed.
- Improvement in abnormally low protein intake relative to the prescribed amount during the initial eight weeks of repeat dosing in the first two patients. Despite the increase in protein intake, patients' plasma arginine values were better controlled with pegzilarginase as compared to baseline values with a protein restricted diet and ammonia scavengers.

“This new data demonstrates that we can objectively measure baseline disease manifestations in patients with Arginase 1 Deficiency using available clinical outcome assessments, including tests of neuromotor function and mobility,” said James Wooldridge, M.D., chief medical officer of Aeglea. “It was exciting to see these clinically relevant effects of pegzilarginase on mobility in the first two patients who had achieved sustained lowering of plasma arginine levels during the eight-week repeat dose part of the study. I look forward to continuing our work, as we believe pegzilarginase has potential as a transformative new therapy for patients with this devastating disease.”

### **Conference Call & Webcast Details**

Aeglea will hold a clinical update conference call today, Thursday, April 12, 2018 at 8:30 a.m. ET. To access the live conference call via phone, please dial 1-877-709-8155 (toll free) within the United States, or 1-201-689-8881 internationally. A replay of the call will be available through April 19, 2018 by dialing 1-877-660-6853 within the United States or 1-201-612-7415 internationally. The conference ID is 13678293.

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 (AEB1102) in Arginase 1 Deficiency**

Pegzilarginase is an enhanced human arginase that enzymatically degrades the amino acid arginine. Aeglea is developing pegzilarginase for the treatment of patients with Arginase 1 Deficiency, a debilitating urea cycle disorder caused by deficiency of a key arginine metabolizing enzyme that leads to severe and progressive hyperargininemia-related neurological abnormalities, hyperammonemia and early mortality. Pegzilarginase is intended for use as an enzyme replacement therapy in patients to reduce elevated blood arginine levels. The Company's Phase 1 data demonstrated that pegzilarginase reduced blood arginine levels into the normal range, supporting its mechanism of action.

## **About Aeglea BioTherapeutics**

Aeglea is a clinical-stage biotechnology company that designs and develops innovative human enzyme therapeutics for patients with rare genetic diseases and cancer. The Company is developing pegzilarginase, its lead investigational therapy, for the treatment of Arginase 1 Deficiency, as monotherapy in arginine-dependent cancers and in combination with an immune checkpoint inhibitor for small cell lung cancer. In addition, Aeglea has an active research pipeline of other human enzyme-based approaches in both therapeutic areas. 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, the timing and success of our clinical trials and related data, 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 Annual Report on Form 10-K for the year ended December 31, 2017 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](mailto:AGLE@investorrelations.com)

## **Investor Contact:**

Charles N. York II  
Chief Financial Officer  
Aeglea BioTherapeutics  
[investors@aegleabio.com](mailto:investors@aegleabio.com)



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