ARG1D Foundation Files Petition Urging Aeglea Biotherapeutics to Reinstate Life-Saving Drug Following Abrupt Withdrawal

ARG1D Foundation Urges Aeglea Biotherapeutics to Reinstate Life-Saving Drug Following Abrupt Withdrawal to Clinical Trial Patients in the United States

SEATTLE, WASHINGTON, UNITED STATES , April 21, 2023 /EINPresswire.com/ -- The Arginase 1 Deficiency Foundation, a leading advocacy group for individuals and families impacted by the debilitating rare metabolic disease, has launched a petition demanding that Aeglea Biotherapeutics Inc. (NASDAQ:AGLE) take immediate action to reintroduce the life-saving enzyme drug, Pegzilarginase,that was abruptly withdrawn from patients enrolled in its clinical trial.

Arginase 1 Deficiency (ARG1-D) is a progressive inherited metabolic disorder that affects patients of all ages, leading to spasticity, missed developmental milestones, intellectual disability, seizures, and early death. The disorder is caused by persistently high levels of the amino acid arginine.

"Arginase deficiency is a devastating neurometabolic disorder. For decades, our standard treatment has not been adequate and leaves children with elevated levels of toxic metabolites that, over time, lead to worsening of their disease. Pegzilarginase is effective in lowering these toxic substances, and patients treated in the clinical trial demonstrated biochemical and clinical improvement. Pegzilarginase is the only hope for children with arginase deficiency to have a better outcome and brighter future." - Angela Sun, MD, FACMG

The drug administered to participants in the Aeglea Biotherapeutics clinical trial demonstrated remarkable efficacy and produced uniformly positive results.

"Prior to this drug, we were forced to watch our children slowly wither away as the current treatment wasn't enough. The previous protocol, consisting of a low protein diet and essential amino acid formula, slowed the progression of the disease but it didn't stop it. Once we were in the trial and on the medication, our children thrived. My daughter's spasticity lessened and allowed her to move easier without battling against her body each and every step, her learning was exponentially quicker, and she no longer had seizures. Now, without this drug, I am seeing all of these terrible symptoms return. Other patients are being hospitalized as their body's are flooded to toxic levels yet again. This terrifying reality was something we hoped was left in the

past." - Tanja Brandt

During the course of a four-year double-blind clinical study, Aeglea Biotherapeutics enrolled 49 participants, nearly half of which were US based, with an approximate age range of 3 to 36 years old. Participants were assured that an Open Label Extension Study would follow, and the drug would be available for another year while the company worked to secure approval from both the FDA and Europe's EMA.

However, the biotech company abruptly ended the study, leaving participants in the United States with no access to the life-saving drug and no explanation.

"Studies in the patients who participated in the clinical trials for the enzyme have benefited greatly. These improvements have included less subjective sense of intellectual dullness and greater independence in the activities of daily living, cessation of seizures, improved ambulation
and diminished spasticity. We would like to see a clear and reasonable pathway for the approval of this “game changing therapy" so that it may be manufactured and made available to the patients who are in such desperate need. The patient community is in despair and needs our help." - Stephen Cederbaum, MD, UCLA

The Arginase 1 Deficiency Foundation, made up of passionate patients and families affected by ARG1-D, has launched a Change.org petition to demand that Aeglea Biotherapeutics immediately take action to reinstate the drug for the benefit of patients nationwide. The foundation aims to raise awareness and garner public support to ensure that the biotech company prioritizes the needs of patients and their families.

Visit their Change.org page to learn more.

Christine Zahn
Arginase 1 Deficiency Foundation
email us here