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CURE HHT BECOMES ONE OF FEW PATIENT ADVOCACY ORGANIZATIONS IN THE WORLD TO SPONSOR A CLINICAL TRIAL OF A SELF-MANUFACTURED INVESTIGATIONAL DRUG PRODUCT

The Cure HHT-sponsored clinical trial of pazopanib to treat HHT-related bleeding has the potential to deliver first FDA approved drug for HHT (Hereditary Hemorrhagic Telangiectasia)

MONKTON, Md., Oct. 09, 2023 — Cure HHT — a nonprofit organization working to fund global research, awareness and education for HHT (Hereditary Hemorrhagic Telangiectasia) — has become one of the few patient advocacy organizations in the world to directly sponsor a Phase II/III clinical trial of an investigational drug product which it owns with the official launch of the pazopanib clinical trial to treat HHT-related bleeding.

HHT is the second most common genetic disease that causes bleeding, affecting an estimated 1.4 million people of all races and backgrounds globally. HHT affects 1 in 5,000 people. It is as common as Cystic Fibrosis and nearly twice as common as Hemophilia, yet there are no FDA-approved drugs to treat HHT.

HHT creates two types of vascular abnormalities, telangiectasias and/or arteriovenous malformations (AVMs), which are fragile and susceptible to rupture and bleeding, and untreated may result in lung and brain hemorrhage, stroke, heart failure and death. The most common symptom of HHT is frequent and severe nose bleeding, which is often dismissed as inconsequential, especially in children.

The launch of the Cure HHT-sponsored clinical trial of pazopanib (pa-zah-pa-nib) at several recruiting sites across the United States is the culmination of years of fundraising, advocacy and bold decision making by the organization after it lost access to the drug in 2015.

Pazopanib is an antiangiogenic therapeutic that is typically given orally to patients with cancerous tumors. When given off-label at a greatly reduced dose than the chemotherapeutic dose, pazopanib appears to show positive effects in HHT patients with outcomes like a reduced need for blood transfusion, iron infusion, and a decreased amount of nose bleeding.

Dr. Marie Faughnan, director of the HHT Center of Excellence at Toronto's St. Michael's Hospital, conducted a multi-center clinical trial in 2015 studying pazopanib to treat HHT-related bleeding at a dose of 50mg. All seven patients participating in the trial showed improvement. At the time, Glaxo-Smith-Kline (GSK) owned the drug product Votrient™ — which is the market name for their formulation of pazopanib. However, GSK sold the drug to Novartis shortly after the trial began and support of HHT-related clinical trials were halted. Thus, the study was closed, and Cure HHT and its physician partners lost access to the promising drug product to use in clinical research.

It was then that Cure HHT made the bold decision to raise the funds necessary to invest in purchasing and manufacturing the drug product to continue the trial on their own.

“Patients deserve approved therapeutics to help treat the chronic aspects of HHT, and they deserve it now,” said Marianne Clancy, Cure HHT’s Executive Director. “It was clear we had to take matters into our own hands if we wanted to make that happen. I am incredibly thankful to our donors who have helped make this groundbreaking effort possible, and to our staff who has worked tirelessly to help us finally reach this point.”

In 2020, Cure HHT successfully applied for and received a $5.2 million grant from the Department of Defense Peer Reviewed Medical Research Program and a $800,000 grant through the FDA to support the trial and finish the HHT clinical trials.

Cure HHT also fought to be granted an orphan drug designation and a breakthrough therapy designation for pazopanib in HHT from the FDA, both of which represented significant milestones in the organization’s pursuit to finally gain FDA approval for the first time for HHT.

"HHT is a not-so-uncommon multisystem inherited bleeding disorder with numerous morbid and potentially fatal complications. The fact that there are still no FDA-approved medications to treat this disease remains a major challenge for people with HHT," said Dr. Hanny Al-Samkari, Associate Professor of Medicine at Harvard Medical School and co-director of the HHT Center of Excellence at the Massachusetts General Hospital. "Given its severity and complexity, we need an armamentarium of effective drugs to treat bleeding and other complications of this disease. The time is now to develop the treatments and cures of the future for this neglected disorder, which will make all the difference for the 70,000 Americans and 1.4 million people worldwide with HHT."

Should the trial show positive results, Cure HHT will apply for a “New Drug Application” with the FDA, where the trial data and results will be evaluated, and hopefully the FDA will grant approval for pazopanib to treat HHT-related bleeding.

To learn more about the clinical trial, please visit CureHHT.org or ClinicalTrials.gov.

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