

FALL  
2023

# NEWSLETTER

## ADVOCACY



## RESEARCH



## COMMUNITY

## AWARENESS

### News & Information for the HHT Community

*Cure HHT is the only organization in the world that advocates for advancements in research, treatment and funding for HHT.*

## Features

**A Monumental Step in the Treatment of HHT**

**Launching a Campaign to End HHT**

**New HHT Centers of Excellence Certified**

**HHT International Scientific Conference Breaks Records**

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The Cornerstone of  
the HHT Community



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## **CONNECT WITH US**



Stay current on HHT clinical trials, education programs and community events. Sign up to receive electronic announcements by registering at [curehht.org](http://curehht.org) or send us an email at [hhtinfo@curehht.org](mailto:hhtinfo@curehht.org).

*Sponsorship and/or advertisement in the Cure HHT newsletter does not constitute endorsement of any treatment or product by Cure HHT.*

# A Monumental Step in the Treatment of HHT

## Cure HHT Becomes One of the Only Patient Advocacy Organizations in the World to Sponsor a Phase II/III Clinical Trial

The **Pazopanib** Phase II/III clinical trial has launched! This is a truly momentous occasion, and reaching this milestone has been extremely hard-fought as Cure HHT is now one of the only patient advocacy organizations in the world directly sponsoring a clinical trial of a drug **we** own. There are currently no FDA-approved drugs to treat HHT, but we are working to change that! Let's reflect on this monumental achievement:

### What is pazopanib and why are new trials needed?

Pazopanib works to stop blood vessel growth (an antiangiogenic therapeutic) and is approved for use in patients with cancerous tumors. When given at a reduced dose, it has had positive effects in HHT patients.

Since clinical trials for pazopanib have not been conducted to completion specifically for HHT patients, it is not approved by the FDA for such use and is therefore generally not covered by insurance.

### How did we get here?

Testing for Pazopanib started in 2015 to treat HHT-related bleeding, and was led by Dr. Marie Faughnan, Director of the Toronto HHT CoE. At the time, the pharmaceutical company GSK owned the drug product Votrient™ (aka pazopanib). All 7 patients in that trial showed improvement! But then, a setback – a corporate buyout of Votrient™ halted the HHT clinical trials and the study closed. We lost access to this promising therapeutic and landed at a crossroads. We could not sit on the sidelines anymore!



We made a bold and unheard-of decision to invest in buying and manufacturing pazopanib ourselves.

With the support of our community, we raised nearly \$1 million to acquire the drug, and secured the resources needed to finish what a \$75 billion pharmaceutical company started. No small feat!

### What goes into gaining final FDA approval?

From applying for funding to writing study protocols and appealing to the FDA, it is no small undertaking. Our tireless work ultimately landed us two FDA designations: orphan drug and breakthrough therapy.

This catapulted our ability to move forward. In 2022, our pursuit of funding resulted in a \$5.2M grant from the DoD, and \$800K from the FDA in support of our trial! These funds were used to build a clinical research organization to oversee clinical sites, fund study operations and more.

If the trial results are positive, and we have high hopes they will be, we apply for a 'New Drug Application' with the FDA, and if approved, work to produce the drug on a larger scale to ensure access for our entire community!

**To learn more about the trial, visit:**  
**[www.curehht.org/clinical-trials](http://www.curehht.org/clinical-trials)**



# Called *to* Cure

**GENERATION TO END HHT**



## Launching a Campaign to End HHT Within This Generation

By Marianne Clancy

The work this foundation has done over the last 30+ years has laid the groundwork for me to be able to finally say – we are close! I am more optimistic and hopeful than I've ever been.

Ending the suffering from this disease in this generation is no longer a pipe-dream. Our tireless efforts over the years to expand our understanding of HHT has put us in a position to leverage rapid advancements in science to end HHT once and for all.

It is no longer a matter of how we can produce better treatments and one day, curative therapies, but truly how quickly we can get it done. The time is NOW!

It's why we are announcing the most ambitious undertaking in our foundation's history! **To accelerate what is possible, we are launching a \$12 million campaign – Called to Cure: Generation to End HHT.**

Enough is enough! It is up to us to get this done. We are calling upon our mighty community to unite so that we may finally end the generational suffering from HHT. This campaign will enable us to catalyze and accelerate advancements within HHT, and attract strategic industry partners to focus on our patient-centered agenda and implementing our *Research Roadmap*.

Your support over the years has helped bring us this close, and your continued support will help us achieve what we once thought was impossible – to end HHT within this generation.



“Immense knowledge of HHT has been amassed. The right philanthropic investors can move things forward rapidly, and there is great hope for this disease.”

**William Sellers, MD**

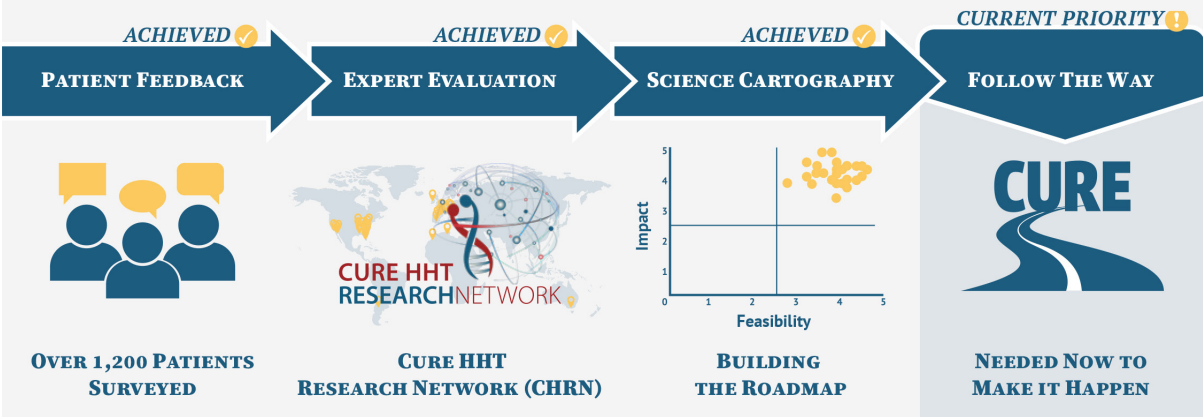
Member of the Broad Institute of MIT and Harvard  
& Director of the Broad Institute’s Cancer Program



Over the next three years, every dollar given to the foundation will be contributed to our **Called to Cure** campaign. This will enable us to:

- Build Out a Cure HHT Therapeutic Arm**  
*Allowing us to directly own the path forward to curative therapies.*
- Fully Implement Our Research Road Map**  
*Our understanding of this disease and the broader scientific landscape is now at a place where better therapeutic treatments and curative therapies are very possible.*
- Strengthen and Grow Our Center of Excellence Program**  
*Expanding access to multidisciplinary care globally, including expansion of pediatric centers.*
- Expand Our Patient Data and Bio Repositories**  
*Groundbreaking discoveries are not possible without the research and ability to expand disease models these provide.*

## BUILDING THE RESEARCH ROADMAP TO CURE HHT



# HHT International Scientific Conference Breaks Records

One of the most meaningful ways we've advanced care for this disease over the years is by sponsoring a biennial HHT International Scientific Conference.

Our fight to drive HHT awareness is only meaningful when we continue to push for progress in science, research, treatment, and care, and these meetings are what bring together the brightest minds across medical disciplines and foster collaboration for the most impactful change for HHT patients!

Over the years, we've significantly grown the number of clinicians and researchers around the world who focus on HHT. There were roughly 50 people at our first HHT International Scientific Conference in 1996.

**At our conference last year in Portugal, there was a record-setting 293 attendees with nearly 50 more joining virtually!** The event had more abstract submissions (209), oral and poster presentations, and young scholar attendees (40) than ever before.

The complete summary of our 2022 conference has been published and is

free to read in *Angiogenesis*—a leading international peer-reviewed journal that is a central source of knowledge for specialists.

Visit the following link to review the articles and gain a better understanding of the scientific advancements ahead:  
[www.curehht.org/resource-library](http://www.curehht.org/resource-library).

We would like to thank the HHT patient community for your voices and continued participation in research—if not for you, the science would not have a straight and narrow way forward.



## Even Bigger & Better Things are Coming in 2024!

Mark your calendars! We are excited to announce our **15<sup>th</sup> HHT International Scientific Conference** will be held in Mandelieu-la-Napoule, France on **October 15-19, 2024**.

*Please stay tuned for more information regarding registration, abstract submission, agenda, and more.*

## Thank You

*A special thank you to our wonderful sponsors this June! Your support allows us to deliver much needed awareness and educational opportunities to our community. We couldn't do it without you!*





# The Long-Awaited Return of our National HHT Patient & Physician Conference

In 2023, we saw the long-awaited return of our in-person National HHT Patient & Physician Conference, previously on hiatus due to Covid-19. Held in June just outside Seattle, the conference welcomed patient and healthcare professionals alike from both North America and abroad! Over 100 patients and family members were in attendance, along with more than 50 medical professionals. We had representation from five different countries!



***“Incredible! It’s still hard for me to believe we patients get to mingle and talk to and ask questions to the docs and researchers. Amazing!” – Patient Attendee***

For the first time, continuing medical education credits were made available to professionals in attendance. This marks a huge milestone in our progress to educate the medical community at large and incentivize them to pay attention to HHT!

HHT patient and physician conferences are so unique because they remove barriers between patients and physicians, and even the red tape between countries and medical institutions. Everyone in attendance is there to learn, ask questions and be a part of the HHT community.

Groundbreaking innovations in research and treatment in HHT were presented; patients and family members spoke directly with the world’s top experts to get answers to their burning questions; elbows were rubbed with the best and brightest in HHT care and research; and pivotal announcements were made! It was an overall amazing experience and we cannot wait to host the next conference. *Mark your calendars! Our next conference is slated for April 6-7, 2024 in the Philadelphia, PA area.*

## New HHT Centers of Excellence Certified



The importance of being seen at a Center of Excellence cannot be overstated. Cure HHT’s ultimate goal is to ensure every patient has access to quality care, and we are thrilled to take another step forward in this goal with the addition of THREE new CoEs – **The University of Miami Health System, The University of Alabama at Birmingham, and Texas Children’s Hospital**. All new centers are accepting patients and welcome your inquiries!



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#GIVING  
TUESDAY

November 28, 2023

## A Message from Cure HHT Executive Director



*Marianne S. Clancy*

Marianne S. Clancy, MPA  
Executive Director, Cure HHT

I've been saying or hearing it over and over again – whether I'm meeting with physicians or pharmaceutical companies, or our team at Cure HHT – ***transformational***. It really is the most fitting word to describe how much is happening within our foundation, and within science and medicine more broadly. I've never been more optimistic, and we've never been closer to real change. Groundbreaking clinical trials are underway, science is rapidly advancing, and transformational leaps have been made in what we know about this disease. What we can achieve in the near future is truly extraordinary.

At Cure HHT, we are determined to capitalize on this momentum by launching our ***Called to Cure*** campaign, building our therapeutic arm to the organization, and implementing our research road-map to drive better treatments and curative therapies now! Of course, none of this is possible without our community. HHT patients everywhere, myself included, are eternally grateful for you!