ADVOCACY / COMMUNITY / RESEARCH / AWARENESS

SPRING / SUMMER 2025 ***NEWSLETTER***

cure hht

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ANNOUNCEMENT: Innovative Hematology Achieves Prestigious HHT Center of Excellence Certification

We are thrilled to share that Innovative Hematology, home to the Indiana Hemophilia and Thrombosis Center (IHTC), has officially earned the coveted designation as an HHT Center of Excellence. This marks another significant step forward in our fight to increase access to specialized care for our community.

This prestigious certification places Innovative Hematology among an elite network of over 30 Centers of Excellence across North America. Each center undergoes a rigorous, years-long evaluation process to ensure these institutions can provide expert care and management of this complex disease.

"Innovative Hematology is excited to partner with Cure HHT in our mission to deliver excellent patient care," said HHT center director Magdalena Lewandowska, MD. "Our HHT center is embedded within one of the largest hemophilia treatment centers in the United States, which is also a medical home. Our patients receive access to a unique multidisciplinary approach, long-term expertise in treating rare bleeding disorders, and many research initiatives. We are located centrally within the state of Indiana, making HHT care more easily accessible."

Innovative Hematology is currently accepting new patients and welcomes inquiries from patients.

To learn more about the new center, please visit innovativehematology.org/hht or Cure HHT's Center of Excellence directory.

PATIENT **RESOURCES**

Navigating a medical condition can be challenging, but it shouldn't have to be. We are constantly collecting and sharing helpful information, tools, and support resources for the HHT community.

See what's new below or reach out to us at hhtinfo@curehht.org for individualized help.

FDA UPDATE: Regarding Iron Infusions

There's been a change to the safety label for Injectafer®, an iron infusion often used to treat iron deficiency. The FDA now includes HHT as a risk factor for developing symptomatic hypophosphatemia (low phosphate levels), a condition that can affect up to 70% of patients who receive Injectafer.

I'VE RECEIVED INJECTAFER INFUSIONS WITHIN THE PAST SIX MONTHS — WHAT SHOULD I DO?

Speak to your primary HHT care provider to ensure they are aware of your situation and can agree on an alternate care plan. It is recommended not to receive any more Injectafer infusions.

Read more at curehht.org/blog.

ACCESS: THE INTERNATIONAL HHT GUIDELINES

Developed by an international panel of HHT experts and patients, the second International HHT Guidelines for Diagnosis and Management of HHT are available for download online. Visit hhtguidelines.org.

ADDITIONAL RESOURCES

• The latest webinars on HHT research, sclerotherapy, and nosebleed management are now available on-demand. Watch them now at curehht.org/webinars.

• NORD recently launched a new initiative designed to help individuals in the rare disease community understand and navigate health insurance. Learn more at claimyourcare.org.

As the cornerstone of the HHT community, we believe in being proactive when it comes to raising awareness, driving research, and providing support to those impacted by this disease.

Looking for more information, programs, or access to the HHT guidelines? Visit curehht.org.

CURE HHT **PROGRAMS**

ADVANCE HHT RESEARCH: Donate Tissue Samples to our New BioBank

By donating tissue samples such as saliva, blood, tissues removed from procedures and surgeries, etc., to our biobank, you can play a crucial role in accelerating HHT research and improving outcomes for patients like yourself. Our biobank is committed to securely collecting, storing, and distributing tissue samples to qualified researchers who are working tirelessly to develop new treatments and therapies for HHT.

Learn more about how you can contribute to this important cause, contact research@curehht.org or visit curehht.org/research/tools-education/donate-tissue.

GLOBAL PATIENT REGISTRY: HHT CONNECT

HHT is a complex disease that affects patients in many ways. The rarity and complexity of HHT makes it particularly difficult to learn about and treat.

That's why we're asking patients to participate in HHT Connect, a global patient registry that looks to equip researchers with data from as many patients as possible so they can better understand and treat this disease.

Visit curehht.org/hht-connect-registry to learn more.

OBSERVATIONAL STUDY: HHT IMPACT

We invite you to join a new study dedicated to understanding the full picture of life with HHT. This fully remote, observational study requires no treatments or travel—just an opportunity to share your experience living with HHT.

By taking part in this study, you'll play a direct role in shaping the future of HHT research, helping to guide future treatments and care that can make a lasting impact for both today's patients and future generations.

Learn more at curehht.org/hhtimpact.

WHAT IS PAZOPANIB?

The story of pazopanib starts more than 15 years ago. In 2009, the pharmaceutical giant GlaxoSmithKline secured FDA approval to use the drug, marketed under the name Votrient™, for treatment of patients with renal cell carcinoma — a rare kidney cancer. Votrient functions as an angiogenesis inhibitor, meaning it helps to prevent the formation and growth of new blood vessels.

When pazopanib was approved to treat kidney cancer, it arrived at a turning point in care—when doctors were shifting from older immune-based treatments to newer drugs that stop blood vessels from feeding tumors. But this wasn't the only way pazopanib would change how diseases are treated.

A NEW APPROACH

By 2015, the success of pazopanib in cancer treatment had sparked new interest in what else the drug might help. Cure HHT assembled a team led by Dr. Marie Faughnan to research whether it could reduce bleeding in people with HHT. Their theory was that since pazopanib prevented the growth of new blood vessels, it might also prevent the formation of blood

A HISTORY OF **PAZOPANIB**

There are no FDA-approved therapeutics for the treatment of HHT. Cure HHT sought to change that.

In many ways, the history of pazopanib is not unlike the story of the HHT community. Both are defined by persistence: a long battle for awareness, funding, and ultimately, solutions. Now, after years of dedicated effort, the paths of pazopanib and HHT have converged, moving forward in unison and resulting in the accomplishment of a pazopanib clinical trial filling and closing to enrollment. This is a major step forward for the future of HHT treatment, as we push to bring our community FDA approved therapies for the first time.

Here's a look back at how we got here.

vessel tangles known as arteriovenous malformations (AVMs) or telangiectasias –hallmarks of HHT and the cause of HHT-related bleeding. Several HHT Centers were already using the drug off label with low dosages and seeing positive results with limited side effects.

Dr. Faughnan and several HHT Center physicians started small, enrolling seven HHT patients in a multi-center project to observe how pazopanib impacted symptoms and quality of life. The results were promising. Over the course of the trial, most patients showed meaningful improvements in both nosebleed duration and severity.

Encouraged by these early efforts, Dr. Faughnan and her study colleagues recommended further and larger-scale research on the potential use of pazopanib as a treatment for HHT bleeding. For a moment, the future looked bright.

But there was a problem.

In the US, drugs must be approved by the Food & Drug Administration (FDA) before they can be marketed and sold for the treatment of a specific condition. That approval hinges on the results of clinical trials, most of which are funded by large drug manufacturers.

When the multi-site trial began, GlaxoSmithKline still owned pazopanib under the name Votrient™. But by March of 2015, GSK had sold much of its oncology portfolio — pazopanib included — to the manufacturer Novartis. And despite the promising results from the initial seven-person study, Novartis declined to sponsor the further trials needed to secure FDA approval for the drug as an HHT treatment.

A BOLD IDEA

HHT, unlike cancer, receives significantly less research funding. While cancer research garners billions annually—one estimate suggesting over $24 billion between 2016 and 2020—HHT remains underfunded. This disparity highlights the critical need to advocate for rare diseases.

Although HHT may not be as prevalent as cancer, it profoundly impacts patients' and families' quality of life. These individuals deserve access to optimal care.

When Novartis declined to pursue promising research that could transform HHT treatment, it put the HHT community at a crossroads. With opportunities like this few and far between, Cure HHT needed to act swiftly and boldly.

A COMMUNITY EFFORT

Since drug owners and manufacturers are responsible for trials, we decided we couldn't just sit on the sidelines. We had to own and manufacture pazopanib — the potential benefits this drug could have on our community was too great. With the support of our remarkable community, we raised nearly $1 million toward our goal and began advocating for additional funding through the Department of Defense and the FDA.

And our efforts paid off. In 2022, we received a $5.2 million grant from the DoD and an additional $800,000 from the FDA to support a trial studying the use of pazopanib as a treatment for HHT. The FDA also awarded us with a Breakthrough Therapy designation, a process that accelerates the review of drugs that have the potential to treat serious conditions more effectively than available therapies.

Since then, Cure HHT has been working tirelessly to build the structure for our clinical research efforts: funding studies, collecting and analyzing data, building protocols and collaborating with partner organizations across statistics, device manufacturing, laboratories, auditing, regulation, and more. We are one of the few nonprofit organizations in the world to be directly running a trial like this. We are relentless in our fight to bring this community better treatment and care.

Now we are one step closer to doing just that, as we announce that our clinical trial has completed enrollment in early 2025! If the results of our trial are successful, we hope to secure FDA approval of pazopanib as a dedicated therapeutic for the treatment of HHT.

This would make pazopanib one of the first FDA-approved HHT therapeutics, providing new care options for those impacted by this disease. FDA-approved treatments are not only much easier to cover through insurance, they're also much more broadly accessible.

Today, the power to deliver affordable, life-changing care is in our hands — and we won't stop until it's in yours.

THE BURDEN OF HEALTHCARE: A Reflection on Cure HHT's Visit with Diagonal Therapeutics

A recent study funded by Diagonal Therapeutics, in collaboration with Massachusetts General Hospital, revealed that HHT presents a significantly higher healthcare cost burden when compared to diseases of similar and greater prevalence.

Using national healthcare claims data, the study team found that HHT ranks as the second costliest condition in a comparison group that included more well-recognized disorders like Crohn's disease, cystic fibrosis, and multiple sclerosis.

KEY FINDINGS

- $500 million: The total annual healthcare cost of those diagnosed with HHT. However, we estimate that only 20% of patients today are diagnosed – so the actual cost is likely much higher.
- Patients who require bleeding support – which represents 54% of our patient population – have an average cost of $40K per patient, per year – making HHT more costly than cystic fibrosis, sickle cell, and muscular dystrophy.
- 11.5% of HHT patients are hospitalized at least once annually.

This study is a game-changing asset in Cure HHT's advocacy efforts, giving us compelling data as we continue our push for increased government funding and to spark greater interest from biotech and pharmaceutical companies. It demonstrates that investing in HHT care and research isn't just the right thing to do —it's smart policy. With the right tools, therapies, and infrastructure, we can dramatically reduce suffering and cost.

On Rare Disease Day, Cure HHT staff had the honor of visiting Diagonal Therapeutics' headquarters to share the lived experiences of our community and emphasize the urgent need for innovative therapies. It was a powerful moment—a rare disease community and an emerging biotech company, standing together in the belief that HHT ***patients deserve better, faster.***

Together, with partners like Diagonal and others pursuing therapies to help this long-overlooked community, we are transforming hope into action.

COMMUNITY **EVENTS**

Our community events are designed to foster learning and connection. Participate in educational webinars, connect with peers, advocate on the hill, or get involved online.

Read below about what we've been up to lately.

SUCCESS ON CAPITOL HILL

In early February, Cure HHT staff members and a team of volunteers met with representatives from across the country to advocate for enhanced funding for key HHT initiatives. Over 50 meetings were held, thousands of steps walked, and the voice of the HHT community was heard.

Our ask was simple: HHT affects 1 in 5,000 people. Every patient deserves access to expert care.

Thank you to those involved — together we can reduce the burden HHT patients face and pave the way to a brighter future! Interested in future HHT advocacy events? Email us at sparkchange@curehht.org.

KNOWLEDGE, CONNECTION, AND HOPE: Recapping Our Regional Conference In Denver

This March, over 160 members of the HHT community came together for a powerful weekend of education, connection, and support. The event featured a Physician Education Day, granting CME credits to more than 60 providers on HHT diagnosis and treatment. Our Patient & Family Day empowered attendees with expert insights and engaging Q&A sessions while kids' programming provided a safe space for those under 18 to connect.

BE ON THE LOOKOUT! *News of our next patient conference will be announced soon.* ***Sign-up for Cure HHT emails to stay up to date on the latest news about this event!***

Discover the latest breakthroughs, inspiring personal stories, and vital updates within the Hereditary Hemorrhagic Telangiectasia (HHT) community right here!

The actions we take today can change the future.

COMMUNITY **UPDATES**

NATURAL HISTORY STUDY: New Study Conducted In Partnership With Diagonal Therapeutics

In March, Diagonal Therapeutics and Cure HHT announced a first-of-its-kind, non-interventional, observational, longitudinal evaluation aiming to characterize the variability of patient-reported outcomes, including epistaxis (nosebleeds), hematologic support, and quality of life (QoL). Read the full press release at curehht.org/blog.

HHT STUDY RECOGNIZED AS TOP CLINICAL RESEARCH ACHIEVEMENT

Join us in congratulating HHT researcher Dr. Keith McCrae! Dr. McCrae was selected as an awardee for the 2025 Top 10 Clinical Research Achievement Awards by The Clinical Research Forum. His study, "Pomalidomide for Epistaxis in Hereditary Hemorrhagic Telangiectasia."

Read about this award at clinicalresearchforum.org.

JUNE IS HHT AWARENESS MONTH

What an incredible June we just had! Our "Beyond the Visible" HHT Awareness Month was a powerful success, all thanks to our amazing community. Together, we truly illuminated the unseen realities of Hereditary Hemorrhagic Telangiectasia.

Stories poured in, bravely detailing the hidden struggles of HHT: the fatigue, the anxiety, the invisible bleeds, and the constant need for self-advocacy. These personal accounts shattered the silence, resonating with countless others and bringing vital understanding to the wider public.

We saw incredible engagement online, with #HHTAwareness spreading far and wide. Webinars, Q&As, and community events strengthened our bonds, providing both education and invaluable support. We've not only raised crucial public awareness, but also empowered individuals within our community, reminding them they're not alone.

This June, we proved that when we unite, the invisible becomes visible, and the unheard finds its voice. Thank you for raising awareness and showing unwavering support.

Marianne S. Clancy, MPA
Chief Executive Officer, Cure HHT

Finding Power And Inspiration Through Community

The connections and lessons we shared in Denver are still resonating deeply with me. Seeing so many of you, feeling the strength of our community, was truly inspiring. It was a powerful reminder of why we do what we do.

We know that the current climate in public health, access to care, and research funding can feel unsettling, and those uncertainties can weigh heavily. Please know this: Cure HHT will not waver. We are deeply committed to advocating for better treatments and a brighter future for everyone living with HHT. Your well-being is our driving force.

We are in this together, and to truly move the needle, we need your active participation. Share your stories, connect with fellow community members, and support our critical research and advocacy efforts. Your involvement, in any form, strengthens our collective voice and accelerates progress.