NEWSLETTER

SPRING 2024

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

Creating Access to Expert Care

HHT Connect – The Global HHT Patient Registry

A Monumental $128 Million Step Forward for HHT Research

Called to Cure: Campaign to End HHT in This Generation (Update)

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Contents

3 Creating Access to Expert Care
Certifying HHT Centers of Excellence

4 Cure HHT Launches “HHT Connect,” the Global HHT Patient Registry
Leveraging rapid advancements in science

You Can Help Expand Physician Knowledge! | Continuing Education Program

New Resource: The Role of Genetics in HHT | Increasing confirmatory genetic testing

5 Federal Funding Protected | $2M secured for HHT Centers of Excellence Program

6 An Update on Our Campaign to End HHT Within This Generation | Fueling breakthroughs – the impact of your generosity

8 A Monumental $128 Million Step Forward for HHT Research
Diagonal Therapeutics focuses on HHT research

9 Key Facts About Our Groundbreaking Clinical Trial | Pazopanib

10 Furthering Knowledge, Building Community, Driving Change
HHT Conferences

11 June Awareness, Breaking News & Leaving a Legacy

12 Message from Cure HHT Executive Director | When yesterday’s dreams become today’s accomplishments

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Sponsorship and/or advertisement in the Cure HHT newsletter does not constitute endorsement of any treatment or product by Cure HHT.
Creating Access to Expert Care

Cure HHT has worked tirelessly to expand access to expert care to more patients. Our goal is to have an HHT Center of Excellence in every state. We are thrilled to announce UC San Diego Health has recently been recertified as an HHT CoE.

UC San Diego Health joins Texas Children’s Hospital, University of Alabama at Birmingham, and University of Miami Health System as four new centers that have been certified by Cure HHT within the last 12 months! To find a center nearest you, visit: www.curehht.org/hht-treatment-center

What does it take to become an HHT Center of Excellence? How are they certified?

It is not a designation we give out lightly! Cure HHT has developed a rigorous process to ensure only centers that are equipped with the knowledge, expertise, and commitment needed to treat this complex and multisystem disease earn the title “HHT Center of Excellence.” To start, let’s take a look at how we got here. The growth in the number of centers is a direct result of our efforts to raise awareness and physician interest in HHT. Establishing more centers is one way we do this, as residents and other doctors at these facilities get exposed to HHT but sometimes leave to work at institutions where HHT is lesser known. We also cultivate the medical community through our scientific conferences, medical webinars, and more.

The physicians we reach may eventually express interest in becoming an HHT Center of Excellence. An application is completed, followed by a several-months training and certification process. Cure HHT examines if the needed expertise is present at the center, a mentor center is assigned, Cure HHT and the mentor center conduct in-person visits and trainings to further educate the prospective new team about HHT, treatment guidelines, and more. Should any concerns or gaps be found during this process, Cure HHT works with the team to address them. A committee then reviews the findings and votes to determine if the institution meets all requirements. Not all applicants make it through to completion.

While we share our community’s want to add as many new centers as possible, it’s our responsibility to ensure that only those with the necessary expertise are named HHT Centers of Excellence.
Cure HHT Launches “HHT Connect,” the Global HHT Patient Registry

Cure HHT is excited to announce the launch of HHT Connect, which is the first global patient registry for HHT. Our goal is to collect health information from as many HHT patients as possible so that researchers and doctors can discover patterns and clues that may help them form a better understanding of HHT, and develop new treatments.

We are now inviting HHT patients from around the world to complete these surveys. By answering some questions about your medical history with HHT, you have the power to play a vital role in giving researchers the data and knowledge they need to work toward life-changing discoveries.

HHT is a complex disease that affects patients in both similar and different ways. The rarity and complexity of the disease make it particularly difficult to learn about and treat. The more patients who participate in HHT Connect, the more we can learn. We need YOU! This is a small way every HHT patient can play a role in creating a brighter future for generations to come.

Learn more about participating by visiting: www.curehht.org/hht-connect-registry

You Can Help Expand Physician Knowledge!

We have recently launched an online platform where doctors and allied healthcare professionals can take courses to further their knowledge in HHT! Participation is FREE, and providers receive continuing education credits when they complete a course!

Our ask to the community is to share this news with your local providers! Learn more by visiting: www.curehht.org/hht-continuing-education-program

New Resource: The Role of Genetics in HHT

Check out our new brochures that outline the important role genetic testing plays in the HHT patient journey. Our hope is for more providers to recognize the signs and symptoms of HHT, and refer suspected cases for confirmatory genetic testing. We have a patient version and one geared more toward providers.

We encourage you to share the provider brochure with healthcare professionals unfamiliar with HHT!

Printable brochures: www.curehht.org/genetics-of-hht

The HHT Continuing Education Program is fully supported by the Health Resources and Services Administration (HRSA) of the U.S. Department of Health and Human Services (HHS) as part of an award totaling $5,862,638 with 0 percentage financed with non-governmental sources. The contents are those of the author(s) and do not necessarily represent the official views of, nor an endorsement, by HRSA, HHS or the U.S. Government.

The Role of Genetics in HHT provider brochure is fully supported by the Health Resources and Services Administration (HRSA) of the U.S. Department of Health and Human Services (HHS) as part of an award totaling $5,862,638 with 0 percentage financed with non-governmental sources. The contents are those of the author(s) and do not necessarily represent the official views of, nor an endorsement, by HRSA, HHS or the U.S. Government.
Federal Funding Protected – But We’re Pushing for More

As a result of our rigorous advocacy efforts, we successfully protected our federal funding to support our HHT Center of Excellence Program. The appropriations bill signed into law by President Biden this spring included $2 million for HHT.

Let’s look at what this means, what’s next, and what we’ve accomplished:

WHAT YOU NEED TO KNOW ABOUT THIS FUNDING

• Funds have been distributed to 15 CoEs, elevating and standardizing multidisciplinary HHT care.
• Future funding is not guaranteed! We have to protect it every year.
• Our ultimate goal is to fund every center!
• Cure HHT does not receive this money! We’re responsible for distributing and overseeing how the money is used, but we are not directly supported by this program.

What We’ve Accomplished So Far

• Created internal team at Cure HHT to serve as the “National HHT Center,” providing guidance, oversight, and coordination to meet program objectives.
• There has been a 44% increase in new patients seen at CoEs across the country, resulting in the prevention of countless tragedies!
• Launched a national data collection registry of HHT patients using deidentified/anonymous data, allowing scientists to track long-term HHT trends to inform breakthroughs.
• Launched an online HHT Continuing Education Program where medical providers can take courses to further HHT knowledge. (See page 4 for link to learn more)
• Distributed “The Role of Genetics in HHT” brochures to providers to educate on the importance of genetic testing in HHT treatment and care. (See page 4 for link to learn more)

What We’re Striving Toward

Our dream is to fund every center in the U.S. To get there, our staff and a passionate group of community advocates took to Capitol Hill in early March 2024. We met with dozens of congressional and senatorial office members and staff, and held a congressional briefing in the Capitol Building. The goal is to increase the program by $1 million in FY 2025.
An Update on Our Campaign to End HHT Within This Generation
By Marianne Clancy

As you have heard, we are in the midst of the most ambitious undertaking in our foundation’s history — a transformational $12 million campaign, Called to Cure: Generation to End HHT.

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.

Called to Cure
Donations Turn Into Life-Saving Impact

What We’ve Accomplished
Funding Curative Therapy Research

Cure HHT is directly funding a research project at the Broad Institute of MIT and Harvard, where they are developing a targeted pathway therapy to prevent the formation of AVMs. Potential antibody candidates will be investigated over the next year before moving to the next phase of testing.

Fueling Breakthroughs in Research

Our scientists have told us a biorepository is essential to fuel research and breakthroughs. So, we’re making it happen. We have launched the first ever HHT biorepository, whereby HHT patients can donate tissue removed during surgery to be studied by researchers across the country. These tissues, DNA and blood samples will be available to any researcher in the world!

Palmer Family Fund for Young Scholars

Thanks to the generosity of Drs. Sara and Jeffery Palmer — both lifelong and passionate advocates for HHT – the Palmer Family Fund for Young Scholars created a fund to provide travel awards for young scholars to attend our scientific meeting, as well as small research grants for early career investigators whose work has the potential to advance HHT treatment.

It is no longer a matter of if we can produce life-saving therapies and curative treatments, but how quickly we can get it done. I wanted to update you on the campaign and share more on the impact we’ve already made thanks to your generous support!

Remember, this is a comprehensive campaign — meaning every dollar raised over the next three years will be contributed toward this effort. Every gift, no matter the size, has impact.

Why $12 million exactly? A convening of the world’s top physicians, researchers, and patients have mapped out projects that will make the most impact, and this is the figure needed to get those projects done. However, we are not waiting until we’ve reached our full goal to get to work. This is far too urgent of a mission!

To date, we have raised nearly $3 million. Although we still have a ways to go to reach our goal, we have already begun work on several targeted projects that will have a profound impact on our community. For once and for all, we can actually end this, but it’s going to take all of us!

“IT is no longer a matter of if we can produce life-saving therapies and curative treatments, but how quickly we can get it done.”

Marianne Clancy, MPA
Executive Director Cure HHT

What’s to Come in 2025

Updating International Care Guidelines

The HHT treatment landscape is rapidly evolving. In 2025, we will be bringing together the world’s HHT experts to update our internationally followed treatment guidelines to reflect changes in PAVM care, pediatrics, women’s health issues, pulmonary hypertension, and more. This will save lives and improve outcomes.

Clinical Fellowship at Mass General Hospital

Thanks to the support of a generous donor, we are launching a fellowship program at MGH, enabling a fully immersive, 12-month experience to a junior faculty member to help induct up-and-coming physicians into an academic career focused on HHT. Our goal is to expand this program across our HHT Centers of Excellence.
A Monumental $128 Million Step Forward for HHT Research

The recently announced launch of Diagonal Therapeutics – a new biotechnology company backed by $128 million in Series A financing – represents a truly monumental breakthrough for HHT, and has the potential to offer hope for every patient impacted by this disease. We wanted to share more about why this is news you must know!

Diagonal’s sole focus is developing an innovative treatment not just to alleviate the symptoms of HHT – but using an antibody treatment to reverse the formation of vascular malformations altogether. Their goal is to have this therapy in a Phase I clinical trial in as little as a year. Yes, you read that right; a company supported by $128 million dollars of funding is focused ENTIRELY on a curative HHT therapy.

For more than a year, Cure HHT has been working alongside Diagonal’s management team to help make this a reality. We have connected them to leading HHT scientists to help further develop their program, and have spent countless hours meeting with venture capital firms to educate key players on the burden of this disease to help Diagonal secure funding to launch.

Learn more about Diagonal and their program by visiting: www.curehht.org/diagonal-therapeutics-announcement

“Cure HHT is so much more than a patient advocacy organization. Nearly two years ago, our organization invested in creating a Therapeutic Development Arm, bringing the necessary talent in house to help us proactively bring forward promising therapies to patients faster. We are not sitting on the sideline and hoping change comes. We are rolling up our sleeves and making it happen. A day like today is a significant milestone for our disease, and represents another reason for our patient community to have hope!”

– Marianne Clancy, Executive Director Cure HHT

Diagonal’s Groundbreaking Approach

Diagonal’s pioneering approach harnesses the power of agonist antibodies going beyond symptom management to potentially alter the disease’s trajectory by reactivating the body’s natural healing pathways. With a blend of bold innovation and technological prowess, Diagonal is setting a new standard for what’s possible in treating HHT.

Going Beyond Symptom Management

Addressing HHT at its very roots, potentially altering the disease’s trajectory by focusing on reactivating the body’s natural pathways towards healing.

Innovative DIAGONAL Platform

The DIAGONAL platform combines advanced computational techniques with experimental science, swiftly pinpointing effective treatments, turning possibility into reality.

Precise Therapies

Diagonal’s treatments are designed to specifically target HHT and specific biological signals, minimizing side effects and maximizing potential benefits.
Key Facts About Our Groundbreaking Clinical Trial

By now, you’ve hopefully heard HHT Centers of Excellence in every region of the country are recruiting for a groundbreaking clinical trial within HHT – the Pazopanib study.

Who should consider participating? What’s involved? And why is this so important? This aims to answer those questions. For full details, head to www.curehht.org/clinical-trials.

WHO (should consider participating): Do chronic nosebleeds impact your life? This may be right for you. This study is open to people with HHT in the U.S. (age 18 to 85 years) with nosebleeding at least 20 min per week with mild anemia OR average hemoglobin <10.

WHAT (is involved): Two in-person visits to a participating HHT Center of Excellence is required, BUT stipends will be given based on miles traveled.

EVERYONE participating has a chance to get the drug. Yes, this is a randomized and double-blinded study, so some patients will receive placebo. However, there is an extension phase. Meaning, even if you received a placebo during the first 24 weeks of the study, you can elect to advance to the extension phase where everyone gets Pazopanib for 24 weeks at no cost.

WHY (is this so important? Where do we begin?):

1) Our goal is that Pazopanib becomes the first FDA approved treatment for HHT. That will make this drug accessible for many patients by making it more likely to be covered by insurance.

2) We need treatment options. Yes, many patients are currently taking off-label cancer drugs to treat their nosebleeds. But the reality is some of those drugs don’t work for everyone. And in other cases, treatment becomes less effective over time.

3) This is the first ever HHT trial funded by the U.S. Department of Defense. Successful recruitment for this trial will improve our chances for future funding for HHT research and trials.

4) This is a hereditary disease. Participating may do more than help you manage your nosebleeds, it has the chance to help generations to come within your own family.

Interested in learning more? Contact research@curehht.org
Earlier this spring, Cure HHT hosted its annual National HHT Patient & Physician Conference just outside Philadelphia, PA. More than 130 patients and 70 healthcare professionals came together to connect and learn about the latest advancements in HHT treatment, research and care. We even had patients attend from Australia and Scotland!

This was the second year we offered continuing education credits for healthcare professionals who attended as a way to further incentivize the medical community to learn more about HHT and the complexities of properly managing the disease.

From stage, groundbreaking innovations in research and treatment were presented. But these conferences are so unique not only because patients can build connections and community with other patients, but they can also rub elbows with the world’s leading physicians and researchers to get their questions answered.

This year, we also had on-site blood draws for attendees to donate to our recently launched Cure HHT BioBank! This will allow researchers from around the world to study real patient tissues and samples, which is critical to advancing treatment.

**STAY TUNED!** Be sure to subscribe to our emails so you’re the first to know about our next conference.

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**15th International Scientific Conference**

Our scientific conference brings together hundreds of the world’s leading physicians and researchers, providing them a space to learn about progress happening across the world and to collaborate in order to fuel future advancements.

This year’s conference is set to take place from October 15-19, 2024 in Madelieu-La-Napoule, France. While the program is geared toward healthcare professionals, patients are welcome to attend.

**Learn more at:** www.science.hhtconference.org
Gear Up for June Awareness Month:

**Become a Changemaker**

In 2009, Cure HHT successfully lobbied Congress to pass a resolution naming June National HHT Awareness Month – dubbing June 23rd Global HHT Awareness Day. This year, we will be asking our community to make the pledge to become an HHT Changemaker.

Stay tuned for resources and more information on how you can partner with us to make a difference in the trajectory of this disease.

**Ensure You Are the First to Know Breaking HHT News!**

As you’ve read in these pages, there is so much exciting news about breakthroughs in HHT research and treatment! But the only way to ensure you know the latest is by subscribing to our emails. We regularly share breaking announcements about transformative new research, new resources, advancements in treatment, information about clinical trials and so much more.

If you have unsubscribed in the past, please consider re-joining us today! If you haven’t already signed up for our emails yet, then what are you waiting for? Add Cure HHT to your address book today and **scan the QR code to connect!**

**Leave a Lasting Legacy Through Planned Giving**

One of the most powerful ways to contribute to our mission is through planned giving, such as including Cure HHT in a will or living trust, establishing a life-income plan, or naming us as a beneficiary in an insurance policy.

Community members who have remembered Cure HHT in their estate plans join an honorary group we call “The Cornerstone Society,” a membership that signifies our warm appreciation of your lasting contribution. To learn more, email: Mary Leigh Krock at maryleigh.krock@curehht.org

Never underestimate the power of your voice!

Simply put, the work we do and all we’ve accomplished would not be possible without the generous contributions of our community! Gifts of all sizes help us prevent tragedies.
I’ve never had hope like this! Yes — over the years our foundation has created so much progress in how this disease is understood and treated, and we have accomplished many significant milestones over the years, but there has never been a time like the present.

Our journey has been a long one, from releasing the patents to make genetic testing possible for patients, to tripling the number of new centers, developing internationally accepted treatment guidelines, funding research and more. Finally, it feels like we can do this! We can end the suffering from HHT, and that’s no longer a pipedream. Curative therapies are being studied. This is real.

Two years ago, we announced the formation of a Therapeutics Development Arm of our foundation to help us more proactively drive progress. These efforts have helped us cultivate new relationships with the biotech and pharmaceutical industry that have the potential to change the trajectory of this disease. We have reached a place where yesterday’s dreams are becoming today’s accomplishments.

I am incredibly thankful to this community for helping us reach this point. But now it’s going to take all of us to reach our ultimate goal. Together, we can be the generation that ends HHT!