Cure HHT
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INSIDE THIS EDITION

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2025 HHT PATIENT AND PHYSICIAN NATIONAL CONFERENCE

NOVEMBER 8-9, 2025 • DALLAS, TEXAS

INVITATION: 2025 HHT Patient and Physician National Conference

I'm so excited to introduce myself to this amazing community. My name is Hellen Tecle, and I stepped into the role of Manager of Community Events and Education here at Cure HHT just a little over four months ago. In that short time, I've been amazed by the strength of this mighty organization, and even more astonished by the resilience, courage, and warmth of the HHT community.

Every day, I'm getting a crash course in just how complex and dynamic HHT is—not only as a disease, but also in terms of the medical, research, and care landscape that surrounds it. There are so many moving pieces, from groundbreaking research to rapidly evolving clinical care to the personal journeys of patients and families. It's become so clear in my short time here: When it comes to HHT, knowledge is power.

It's with that in mind that I'd love to personally invite all of you to join us for our 2025 National HHT Patient & Physician Conference. This special gathering will take place just outside Dallas, Texas on November 8-9, and it promises to be an unforgettable experience… filled with warmth, hope, empowerment and more!

Whether you've been newly diagnosed, or your family has been navigating HHT for generations, this conference is for you. Maybe you've never attended before, or perhaps it's been a few years since you last came—either way, I want to encourage you to take this opportunity to take charge of your care.

The program will feature some of the world's leading HHT experts who are at the forefront of research, treatment, and patient care. It's rare to have such a wide array of specialists in one place, ready to share their knowledge and answer questions directly. Since we launched registration, our inbox is filled with emails from past conference attendees saying how the information they gained at these events made a real and life-changing difference in how they manage their disease.

But equally important is the sense of community you'll feel. There is something powerful about being in a room filled with people who truly understand the challenges and triumphs of living with HHT. Families share their stories, patients encourage one another, and connections are made that last long after the conference ends. It's a reminder that none of us are walking this path alone.

Our conferences are also designed to be uplifting and practical. We'll cover a wide range of topics, from medical breakthroughs to everyday strategies for living well with HHT. You'll gain the knowledge and tools you need to advocate for your health, support your loved ones, and feel empowered in your journey.

When we come together, we create something extraordinary: a community that carries both the science of tomorrow and the compassion of today. I can say with confidence that this conference will leave you inspired, informed, and connected in ways that are deeply meaningful.

I truly hope to see you in Dallas in November. Let's join hands, share stories, and fuel the fight for better treatments and, one day, a cure.

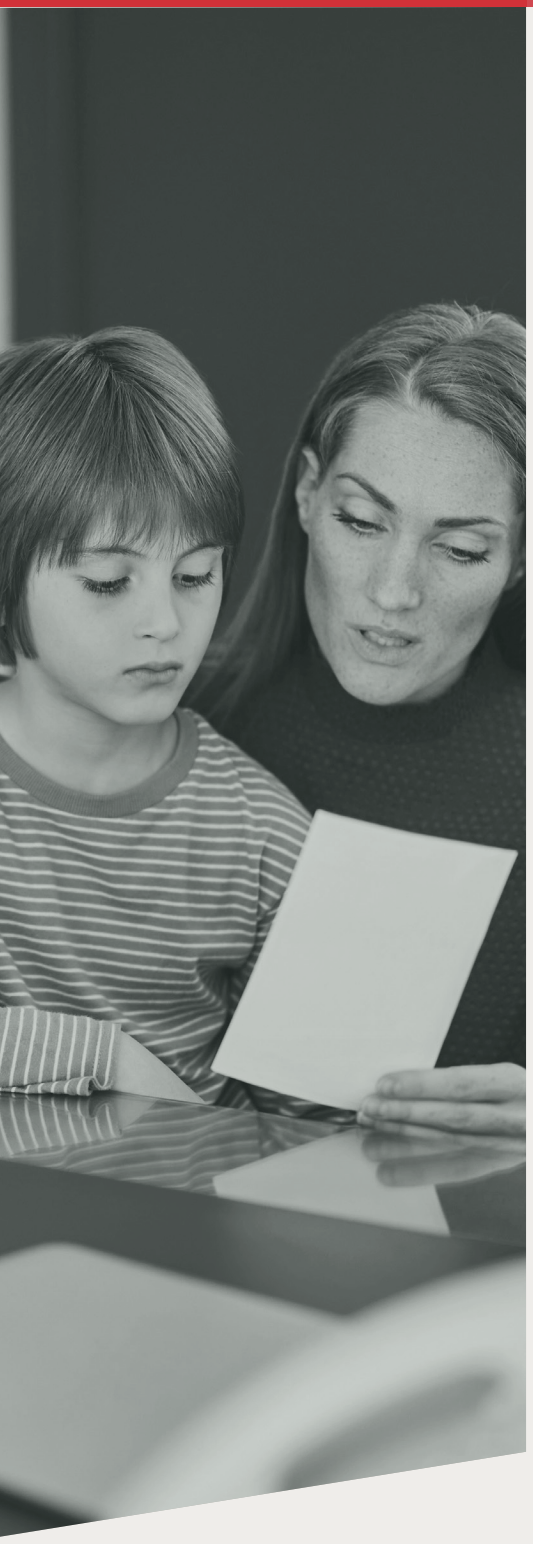
HELLEN TECLE
Manager,
Community Events and Education

Join us in Texas:
curehht.org/national25

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.



CONSENSUS REPORT: Advancing Research and Care

A recently published international consensus report—led by Cure HHT and published in the American Journal of Hematology—establishes the first global standards for evaluating bleeding in HHT. Affecting 1 in 5,000 people worldwide, HHT is the second most common inherited bleeding disorder.

The report introduces clear criteria for bleeding severity, treatment goals, and two new measures—the Hematologic Support Score (HSS) and Hematologic Impact Score (HIS)—that better capture the impact of chronic blood loss. These standards will strengthen clinical trials, speed drug development, and improve patient care.

"This is a foundational step forward for HHT research and patient care," said lead author Dr. Hanny Al-Samkari.

Read more at ***curehht.org/blog***

Get Support: The Cure HHT Website Offers Tools & Rersources

Cure HHT offers resources to help patients and families access expert care and information. From HHT Treatment Centers and a "Find a Doctor" tool to educational materials, webinars, and international support networks, you can connect with the right guidance at every step of your journey.

Check your Nosebleed Severity Score: ***hhtess.com***

WATCH IT NOW: Cure HHT Webinars

The latest webinars on managing your mental health, HHT research, and nosebleed management are now available on-demand: ***curehht.org/webinars***.

Add a recording to your watch list: ***curehht.org/webinars***

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 RESEARCH

Your participation makes a difference. By joining one or more of these initiatives, you can help researchers collect the information needed to improve treatments, accelerate discoveries, and bring us closer to a cure.

HHT BioBank

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***

HHT CONNECT

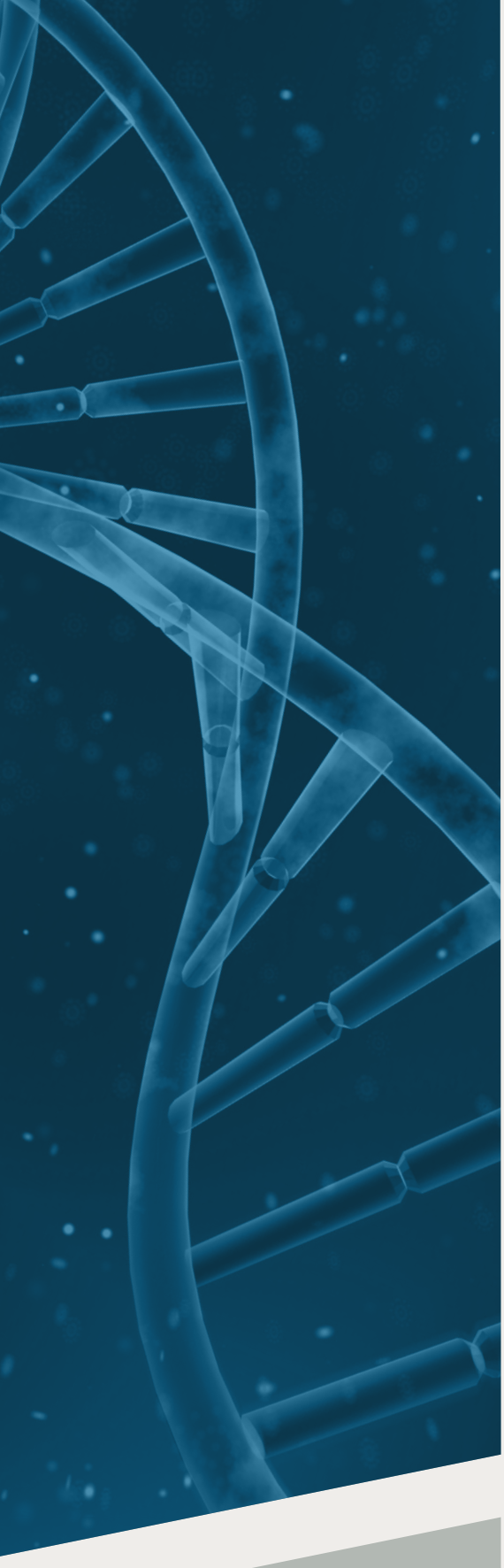
We're asking patients around the world to participate in HHT Connect, a global patient registry that looks to equip researchers with data from as many patients as possible so they can better understand and treat this disease.

Visit ***curehht.org/hht-connect-registry***

CHORUS

We invite you to learn more about "CHORUS," our patient outcomes registry. Our goal is to have more than 10,000 patients participate in this survey, which will allow us to follow a large cohort of patients over time—helping us better understanding the impact the disease has, and the factors that influence them.

Learn more at ***curehht.org/chorus***



HHT: A **WHOLE SYSTEM** DISEASE

HHT is often introduced to the world through a single symptom — nosebleeds. But HHT is never only one thing. It is a whole system disease, one that touches organs, families, and futures.

More Than a Nosebleed

For decades, HHT was mischaracterized as a rare bleeding disorder, defined only by the recurrent nosebleeds that so many patients endure. But the truth is far broader, and far more urgent. HHT is a disease of the vasculature, affecting blood vessels throughout the body.

Telangiectasias on the skin and in the GI tract, arteriovenous malformations (AVMs) in the lungs, brain, and liver — these are not isolated features. They are a reflection of the systemic nature of HHT.

> *"People used to say, 'Oh, you just have nosebleeds,'" recalls Angela, who has HHT. "But when I learned I also had AVMs in my lungs, everything about how I understood my body changed. It wasn't just an inconvenience — it was a disease that could affect every part of me."*

To call HHT a "whole system disease" is to acknowledge that every organ is a potential site of involvement. It is to recognize that the heart, lungs, liver, and brain can be drawn into the story, sometimes silently, sometimes catastrophically. And it is to underscore the importance of a comprehensive approach to diagnosis, treatment, and long-term care.

The Diagnostic Odyssey

For many families, the journey begins long before the words Hereditary Hemorrhagic Telangiectasia are ever spoken. A child faints at school. A mother endures years of iron infusions without explanation. A grandparent is told their liver disease

must be alcohol-related, despite no history of drinking.

On average, it takes nearly three decades from the onset of symptoms to reach a diagnosis. This odyssey extracts a toll — not just in medical outcomes, but in emotional resilience. Families find themselves navigating uncertainty, balancing hope against fatigue, and carrying the weight of knowing "something is wrong" without the relief of a name for it.

> *David remembers the decades before his diagnosis: "I thought I was just unlucky. I had nosebleeds, my daughter had them, my mom had them. Doctors treated us separately, but no one connected the dots. It wasn't until my granddaughter needed blood transfusions at age eight that someone finally said, This could be genetic. That moment changed everything."*

Cure HHT has long recognized that closing this diagnostic gap is essential. By equipping physicians with education, expanding the network of HHT Centers of Excellence, and advocating for broader genetic testing, we are transforming the diagnostic journey from a decades-long maze into a clear, navigable pathway.

The Family Thread

HHT rarely affects just one person. It is hereditary, weaving itself through family trees, sometimes quietly, sometimes unmistakably. To live with HHT is often to live within a network of relatives — some symptomatic, others asymptomatic, many still undiagnosed. Others choose to avoid their diagnosis altogether.

This brings a unique dimension to the disease: the conversations around testing and screening children, the choices parents make about disclosure, the siblings who wonder what the gene means for them.

> *Leah, 29, shares: "My dad didn't want to get tested, even after I was diagnosed. He said he didn't want to 'know too much.' But I needed to know for myself, and for my future kids. It's complicated, because it's not just your health — it's your family's story too."*

And then there is aging. For older adults with HHT, the disease intersects with the vulnerabilities of later life: increased fragility, higher surgical risks, and the need for coordinated care across multiple specialties. The system-wide nature of HHT demands not just a pediatrician or a hematologist, but a team — and often, a family advocating persistently at their side.

The Social Side of Medicine

Medicine is never practiced in a vacuum. HHT patients navigate not just physical complications, but the social ripple effects of chronic disease. Children who miss sports tournaments for hospital visits. Teens who wonder how HHT will shape their futures. Adults who juggle work, family, and the unrelenting logistics of care.

> *Carlos says: "I just wanted to play soccer. But between nosebleeds and hospital trips, I missed half the season. My friends didn't get it. They thought I was exaggerating. Sometimes I didn't even know how to explain it."*

Even the most organized among us — with color-coded notes and bento-box precision — feel the strain of coordinating multiple specialists, insurance approvals, and urgent procedures. The weight of uncertainty presses hardest in moments that should be ordinary: school trips, career milestones, or family celebrations.

Cure HHT's role is not only to advance medicine but to build a community that validates these experiences. Support groups, patient education, and advocacy work are as critical to well-being as clinical trials. By giving patients and families a platform to share their voices, we create a collective strength that carries individuals through the hardest moments.

An Ecosystem Approach

If HHT is a whole system disease, then the response must also be whole-system. No single researcher, physician, or family can carry the burden alone. What is required is an ecosystem — a network of clinicians, scientists, advocates, and patients working in concert.

Cure HHT has positioned itself as the hub of this ecosystem. We connect the dots: from patients to providers, from laboratories to living rooms, from government agencies to grassroots fundraisers. By

aligning these moving parts, we accelerate progress that no one branch could achieve in isolation.

In research, this means driving discovery in vascular biology while ensuring findings translate quickly into therapies.

In care, it means supporting Centers of Excellence and expanding training so that every physician can recognize HHT, not just the specialists.

In advocacy, it means securing funding, shaping policy, and elevating the voices of patients until they can no longer be overlooked.

In community, it means standing with families through diagnosis, treatment, and the countless untold challenges of daily life.

The Path Forward

To live with HHT is to live with complexity — but also with extraordinary resilience. Each story in this community is a reminder that while the disease affects the whole system, it does not define the whole person.

Our mission at Cure HHT is to ensure that no one faces this journey alone. Through science, advocacy, and the power of community, we are working toward a future where HHT is diagnosed early, treated effectively, and ultimately cured.

Because when the whole system is engaged — science, medicine, policy, and people — the whole system can change.

Together, we're building a future where HHT no longer stands in the way of life.

VALAREE MACHEN
Senior Manager,
Marketing and Communications

Share your HHT experience with us:
curehht.org/share-your-story

PATHWAYS TOWARDS SUCCESS

Cure HHT takes pride in playing a vital role in progress by building genuine relationships with patients, physicians, researchers, policymakers, and industry partners. All roads lead to Cure HHT. That's why we are the cornerstone of the community, driving collaboration that brings life-changing progress closer every day.

Email us directly at ***hhtinfo@curehht.org***

COMMUNITY **UPDATES**

Discover the latest breakthroughs, inspiring personal stories, and vital updates within the HHT community right here!

The actions we take today can change the future.

BACK-TO-SCHOOL SEASON IS HERE

As a new school year begins, it's important to make sure children living with HHT are informed, supported, and prepared. Talk with teachers, school nurses, and staff about your child's needs, from managing nosebleeds to recognizing signs of anemia. Share Cure HHT resources so educators feel confident in providing care. With preparation and communication, kids can focus on what matters most—learning, friendships, and growth—while parents have peace of mind knowing their school community is ready to help.

Access resources in our video library:
curehht.org/patient-topic/hht-in-children

CALLED TO CURE: Now Is Our Moment

We launched our Called to Cure Campaign nearly two years ago because science is evolving so quickly and our understanding of the underlying mechanisms of HHT has finally reached a place where seismic leaps in treatment are possible. Curing HHT is no longer a distant dream. It can be done. The only question is when, and how quickly we can make it happen.

Through this campaign, Cure HHT is driving unprecedented momentum. We now have 20 therapies in the pipeline, including several with the potential to reverse AVMs, and groundbreaking research projects are underway to address critical needs in women's health, pediatrics, heart and liver complications, and more. These advances make it clear: we have the tools, talent, and partnerships needed to transform the future of HHT.

Thanks to the generosity of our community, we've already raised $8.4 million toward our $12 million goal. This support has allowed us to expand clinical trials, forge biotech partnerships, and build a therapeutic division at Cure HHT dedicated to accelerating this work. But to keep that momentum alive, we need the full strength of our community to step forward.

Together, we can achieve what once felt impossible: to cure HHT within this generation. Now is the time.

Learn more ***curehht.org/called-cure***

ADVANCES IN RESEARCH AND CARE:
Recently Published Studies

Over the past few months, the HHT research community has made remarkable strides across a wide spectrum of discovery. From case studies that expand our understanding of rare complications, to reviews and consensus papers shaping global standards, to cutting-edge science uncovering disease mechanisms and clinical markers, each publication contributes to advancing care and accelerating the path toward new therapies.

Healthcare Costs and Utilization

A large U.S. claims analysis found that people with HHT face exceptionally high healthcare costs—averaging $19,000 per year for those with mild symptoms and $40,000 per year for those requiring hematologic support.

Standardizing Outcomes in Research

An international consensus paper underscored the need to standardize outcome measures in HHT research, ensuring that new treatments can be fairly tested and compared across studies worldwide. In an accompanying editorial, Dr. Magdalena Lewandowska praised this effort, writing:

> *"The Global Research and Medical Advisory Board and the Cure HHT Foundation should be commended for their leadership and vision. The standardization of terminology and outcomes in HHT marks a turning point in the field – one that promises to accelerate innovation, improve care, and bring us closer to a cure."*

Brain Vascular Malformation Biomarkers

As the 16-year Brain Vascular Malformation Consortium concluded its funding, a new study examined plasma samples from patients with brain vascular malformations (VMs) and healthy volunteers. The research explored circulating biomarkers that could one day help monitor disease activity and therapeutic response. While further work is needed before clinical use, this represents an important step toward precision medicine and reducing reliance on imaging in HHT and related conditions.

PIEZO1 as a Therapeutic Target

Dr. Anne Eichmann's lab identified that PIEZO1, a mechanosensitive ion channel, is overactive in blood vessel cells lacking ALK1 (as in HHT2 caused by ACVRL1 mutations). In mouse models, blocking or removing PIEZO1 reduced vascular malformations, highlighting it as a promising therapeutic target for a significant subset of HHT patients.

SMAD4 and Aortic Complications

A case study by Dr. Marcelo Serra and colleagues reported on a patient with HHT due to a SMAD4 mutation, which also causes juvenile polyposis. Tragically, the patient died from an aortic dissection. A literature review conducted by the team supports the need to screen HHT-JP patients for aortic anomalies as part of standard care.

Questions? Reach out to us at:
research@curehht.org

WELCOMING NEW FACES: Expanding Our Impact

Cure HHT continues to grow — and with it, our capacity to serve the community. Each new staff member brings valuable expertise and passion that strengthen our mission. This growth means more programming, resources, and support for patients, families, and clinicians worldwide. We're proud to introduce the dedicated individuals who are helping drive progress, build connections, and bring us closer to a cure.

Be sure to say hello in Dallas in November at the 2025 HHT Patient and Physician Conference!

ADRIENNE BARTON, CFRE
Chief Operating Officer

TANIA COMPETIELLO
Sr. Director, National Healthcare Programs

KASSIDY SWORDS
Research Coordinator

HELLEN TECLE
Manager, Community Events and Education Manager

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.

MAKING HHT HEARD: In The Halls of Congress

In September, Cure HHT hosted a Congressional Briefing in the historic Kennedy Caucus Room on Capitol Hill, thanks to the sponsorship of Senator Richard Blumenthal (CT-D). Dr. Hanny Al-Samkari delivered a powerful message on behalf of the HHT community, urging Congress to advance research and care. His words underscored the urgency for families living with HHT and made clear that funding this work is simply common sense.

The room was filled with engaged staffers and thoughtful questions, reflecting a growing awareness that HHT deserves attention at the highest levels. Among the attendees were members of the Ribicoff family, relatives of the late Senator Abraham Ribicoff of Connecticut. With five known generations affected by HHT, their presence in a room where Senator Ribicoff once worked was a meaningful reminder of how history and community shape progress.

Our breakthrough came in 2023, when HHT was first recognized in federal research funding. But as this briefing showed, more work remains.

Join us in advocacy—learn how by emailing ***sparkchange@curehht.org***

CURE HHT AT ASH 2025: Our First Symposia

Cure HHT is making history at the 67th American Society of Hematology (ASH) Annual Meeting and Exposition this December. For the first time, our organization will host an official symposia on Friday: *Targeting Angiogenesis and Managing Severe Anemia in Vascular Bleeding Disorders: Hereditary Hemorrhagic Telangiectasia, Von Willebrand Disease, and Beyond*.

Sponsored by Pharmacosmos, this program highlights cutting-edge research and treatment strategies for HHT and related disorders. Having a dedicated platform at ASH — the world's largest hematology meeting — marks a major milestone in raising awareness, advancing science, and accelerating progress for the global HHT community.

SAVE THE DATE: Friday, December 5, 2025

curehht
The Cornerstone of the HHT Community

Imagine living with a disease that has no FDA-approved treatment or cure.

That's the reality for the 1 in 5,000 people around the world who live with HHT.

80% of people affected don't even know they have it yet.

Change the future: ***curehht.org/give***

YES! Please accept my tax-deductible gift to Cure HHT:

☐ $100 ☐ $250 ☐ $500 ☐ $2,500 OTHER $ _____

Make checks payable to:
Cure HHT, PO Box 329, Monkton, MD 21111

Please charge my credit card: ❑ Visa ❑ MasterCard ❑ American Express ❑ Discover

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This is a gift in memory of: _____

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FOLLOW US

Our social media channels are the easiest way to stay informed and engaged. Follow Cure HHT to hear the latest news, learn about upcoming events, and connect with others in the community. Together, every post, share, and comment helps raise awareness and strengthen our movement.

STAY CONNECTED WITH CURE HHT

By following Cure HHT on Facebook, Instagram, LinkedIn, and X, you'll always have the latest updates at your fingertips. From research breakthroughs and advocacy wins to patient stories and event announcements, our channels are designed to keep you connected. It's also a space to amplify awareness—every like, comment, and share helps bring HHT into the spotlight and build momentum for a cure.

Join the conversation and be part of a growing, global community making a difference every day!

/hht.org

@cure_hht

@curehht

@HHTFoundation

THANK YOU

Celebrate the progress that's possible thanks to the generosity of our sponsors! Their support powers research, education, and advocacy efforts that bring hope to the HHT community every day.

Together, we are transforming challenges into opportunities and moving closer to a future free of HHT.

THANK YOU

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SUPPORT PROGRESS THROUGH A PARTNERSHIP

Cure HHT offers partnership opportunities that connect sponsors with impactful programs advancing awareness, education, and research for HHT. With 1.4 million people affected worldwide, support is critical to expand initiatives such as HHT Academy Patient & Physician Conferences, International Scientific Conferences, awareness campaigns, and online resources. Partnerships help bridge the gap between industry and treatment, bringing life-changing progress to the HHT community.

Scan the QR code to read more or email us directly at ***hhtinfo@curehht.org***

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

Victory for the HHT Community

Congratulations to each of you. Thanks to your dedication in our recent *Dear Colleague* campaign, we have reached a pivotal milestone in our advocacy journey. Your calls, emails, and personal stories carried weight in the halls of Congress, and lawmakers are beginning to recognize the urgent need for federal support of HHT research and care.

This is not the finish line—but it is meaningful progress. As legislation advances step by step through Congress, your voices remain the driving force that ensures HHT is heard and prioritized.

As Margaret Mead wisely said, "Never doubt that a small group of thoughtful, committed citizens can change the world; indeed, it's the only thing that ever has." Our community has once again shown the truth of those words.

With momentum on our side, I am confident that together we will continue to break barriers, open doors, and bring new hope to families impacted by HHT.