WINTER
2025

NEWSLETTER

ADVOCACY

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COMMUNITY

AWARENESS

cure hht
The Cornerstone of the HHT Community

Features

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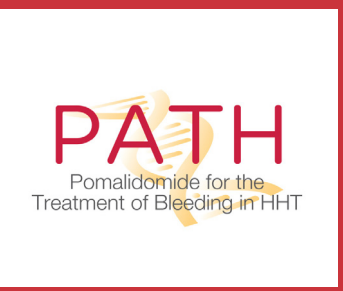
Major Wins For HHT Treatment, Awareness & Research!

Within the last few months, there have been two major announcements in the world of HHT research and treatment. These successes are a testament to our ability to serve as the catalyst for driving HHT science forward – whether by building relationships with industry, growing the research community, directly funding research, or being the bridge that connects research to patients. These milestones brings us one step closer to more effective treatments and a brighter future for all those affected by HHT.

PATH Makes History as the 1st Positive, Large-Scale Trail for an HHT Therapeutic



The National Institutes of Health-sponsored PATH trial is complete, becoming the first positive, large-scale randomized trial for an HHT therapeutic. This study tested the use of the cancer drug pomalidomide in treatment of HHT-related bleeding — and researchers saw very promising results. The data was strong enough that the trial ended early!

But the news gets even better: The study was published in the ***New England Journal of Medicine — one of the most prestigious medical publications in the world,*** read by more than one million physicians every week. This is a huge win for HHT Awareness, as this publication puts HHT in the spotlight of the medical community.

Vaderis Therapeutics Announces First Ever Positive Results for Industry-Led HHT Trial

Earlier this fall, Vaderis Therapeutics announced the successful results of their proof-of-concept trial for HHT. This represents **the first-ever positive clinical trial led by the pharmaceutical industry specifically for HHT**.

The company is developing VAD044, an oral, once daily AKT-inhibitor which represents the first novel therapy intended specifically for HHT treatment. Cure HHT is proud to have played a critical role in encouraging Vaderis to focus on HHT, as well as providing the patient perspective to trial design.

Vaderis also recently announced it has received a "Fast Track" designation from the FDA, which will help expedite the study -- an important milestone!

Recapping our Monumental 15th International HHT Scientific Conference

If there is one takeaway the community needs to know following our scientific conference, it's this: Real change is happening now. Here is the must-know news from our four-day event held just outside Cannes, France – which brought together the world's brightest minds in HHT medicine, science and research to discuss and collaborate around the latest advancements in HHT.

Shattering Records

- Virtually every conference record was broken; We had over 370 attendees, with 29 countries represented. This included 72 trainees – one of the ways we work to inspire the next generation of HHT leaders!

So Much Science

- Records were also broken for the sheer volume of science shared, with 49 oral presentations and 176 poster presentations, showcasing innovative and diverse research presented throughout the event.

Reversing Disease

- Diagonal Therapeutics, a newly launched biotech whose sole focus is HHT, gave several groundbreaking presentations detailing their approach to an antibody therapy that treats HHT at the biological level. This drug can potentially reverse and prevent disease, and they anticipate treatments getting to patients in the next two years!

Therapies not Surgeries

- A major milestone was achieved at this conference: We had our first ever full session focused on randomized trials within HHT. Just four years ago, there were no HHT clinical trials. From stage, the results from five promising trials were shared.

Recognizing Impact

The prestigious Robert I. White Jr. Young Clinical Award – given to recognize outstanding contributions to HHT care – was awarded to Lauren Belsow, MD (Children's Hospital of Philadelphia) for her compassionate work to transform pediatric HHT care.

The Robert E. Berkman Leadership Award was bestowed upon Duke University research leader Douglas Marchuk, PhD, recognizing his 30 plus years of accomplishments from HHT gene discovery, to understanding why HHT affects patients differently in the same family.

2024 Impact Report

A Year of Significant Transformation & Progress

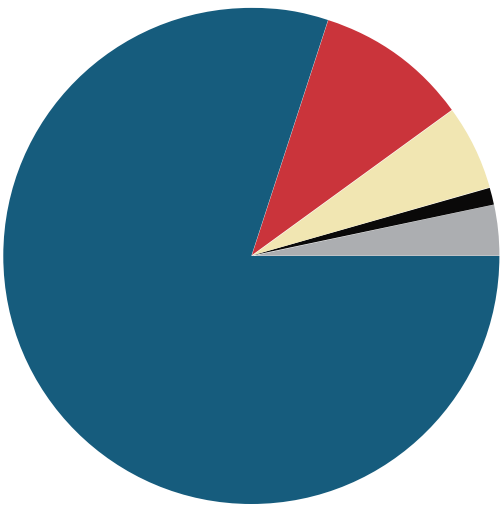
Every year is a different chapter in our story – each bringing us closer to our ultimate goal. But 2024 was defined by new, especially significant, and meaningful results in moving science and research forward. This year, thanks to the efforts of our new Therapeutic Development team, we accelerated HHT research to never-before-seen levels and have increased active HHT therapeutic projects by 300% since 2022. This work is translating into therapies that extend life and reverse disease. Critical breakthroughs in HHT treatment are just ahead. We are the catalyst that drives science forward, and the types of projects underway are those that will have a tangible impact on care and families impacted by HHT.

The projects we're now leading have grown in significance, but our mission remains the same: to save lives and improve well-being of those suffering from HHT on our path to a cure. Research, education, public information and advocacy are still the pillars of everything we do, and none of them can happen without the others.

"The pace of work and progress in this past year has been incredible to witness. We're knocking on the door of outcomes that just a few years ago seemed impossible. We have an unbelievable scientific and medical group committed to these projects, but make no mistake — the support of our HHT community is the reason we're able to do this work."

– Marianne Clancy, Executive Director

Donations are the most critical driver of real-life impact. In 2024, 88% of dollars went directly to program services.



- 73% Research
- 9% Education
- 5% Public Information
- 1% Advocacy
- Management only 3% of total expenses

This past year, we've seen huge returns from our strategic investment in research. On the next page, our impact report further outlines the progress we've driven this year… and the hope that lies ahead.

By The Numbers

Our multi-pronged approach to research turns donations into millions of dollars dedicated to innovative treatments, better technology and more effective treatments.

$1.5 Million
Invested in 28 seed grants

$53 Million
In leveraged investment from government agencies

2,397%
Return on investment

A Therapeutic Approach

This year we doubled down on our therapeutic strategy, driving innovation through partnerships with industry trailblazers and prioritizing a strategic research portfolio ensuring focus on the most meaningful projects for patients.

Key Achievements in 2024

- Introduced HHT Connect, our first ever patient registry
- Launched centralized HHT biorepository to support international research
- Created an online platform to educate physicians about HHT
- Protected federal funding for the third consecutive year, contributing to a 44% increase in new patients seen at Centers of Excellence
- Entered year 2 of our directly sponsored Phase II/III pazopanib trial
- Successfully recruited for the first positive-large scale HHT Study, the PATH Trials – the results of which were published in the New England Journal of Medicine
- Played critical role in helping Diagonal Therapeutics launch with $128M in seed funding, focusing entirely on a curative HHT therapy
- Secured more than 6 active partnerships with biotech and pharma focusing on novel HHT therapies -- all aimed at restoring vasculature to normal and reversing disease
- Funded a grant to the Broad Institute of MIT and Harvard to support development of a compound that has the potential to reverse disease

1,708
webinar and patient conference attendees in 2024

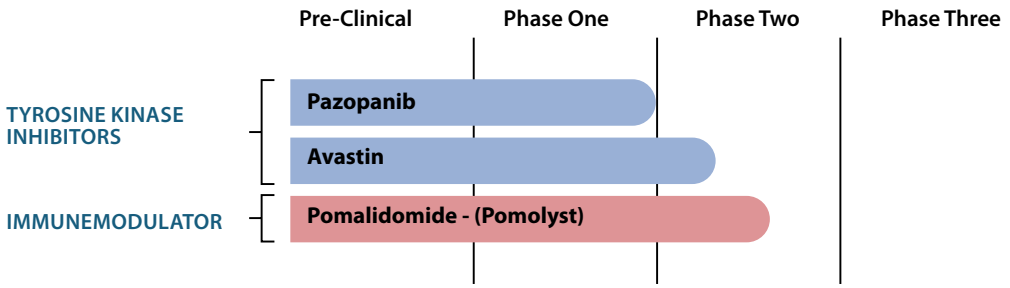
394
CME credits provided to healthcare professionals in 2024

Research in Action

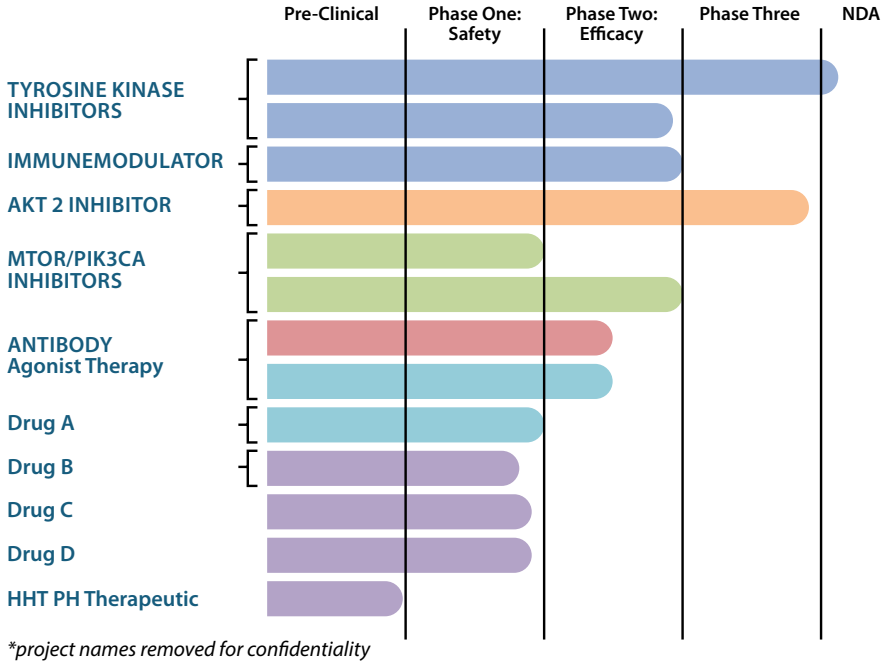
We continue to make enormous strides in the scope of our research. Just three years ago, Cure HHT had no active trials or research projects underway. Today we have more than ever before, with 13 pivotal efforts currently in progress.

HHT Treatment Pipeline: Then & Now

2018-2022: 3 Projects Underway



2024-2028: 13 Projects Underway



**project names removed for confidentiality*

New Frontiers in Publishing

We continue to meaningfully grow the community working on HHT, as evidenced by the explosion of HHT medical publications this past year. This hasn't happened by chance. It's the result of years of effort. **In 2024, we published outcomes from 51 randomized trials** in medical journals around the world. It was our biggest publishing year in our more than 30-year history as an organization and will be instrumental in continuing to bring new attention and work to our cause.

Number of HHT Publications by Year

Remembering the Life, Legacy and Impact of Two Visionary Cure HHT Leaders

By Marianne Clancy

It is with a heavy heart that I share the news of the passing of two of Cure HHT's most influential leaders, advocates, supporters, and friends – Robert "Bob" E. Berkman and Charles "Chuck" Abbott. Both of these men were involved in the foundation from its earliest days, both having served as former presidents of our board of directors. It is beyond difficult to justly summarize their legacy in just a few words. Simply put, the progress we've made today wouldn't be possible without the support of Bob and Chuck.

Under Bob's two terms as Board President, Cure HHT truly evolved to become the cornerstone of the HHT community. He drove the development of our first strategic plan, ensured we were laser-focused on priorities and challenged us to expand access to care across North America. When he joined the board, we had only 8 Centers. Under Bob's leadership, we certified a new center ever year and today have nearly 30 in the US. Bob was the consummate mentor and coach, constantly challenging us to think outside the box and look at the big picture.

Chuck's influence on the growth and evolution of our foundation begins nearly on Day 1. His early leadership, commitment and dedication helped ensure this idea to form an organization to drive research, awareness and treatment could take flight and grow into what we are today. Chuck was one of Cure HHT's earliest supporters, and most dedicated friends. He attended every patient conference, sharing his family's story of significant loss due to HHT with our patient, medical and scientific communities, Chuck was a committed to increase awareness and participated as an active legislative advocate to secure research and HHT Center of Excellence funding. Chuck relished his role as the auctioneer at each national patient conference event – helping to inject fun into our gatherings and raise significant dollars over two decades to fund life-saving change.

We are forever thankful for both of these men, their friendship, and significant contributions to this community. We will miss their wisdom and guidance, but their legacies live on as we continue to change the trajectory of this disease.

Empowering and Connecting our Community: UCSF Kids Day Smashing Success!

We're proud to have recently hosted an HHT Kids Day alongside our partners at the UCSF Center of Excellence at the California Academy of Sciences Museum. Made possible through our HRSA grant, this event saw nearly 70 families – including children of all ages – come together to connect with fellow HHT families and hear from leading experts on topics ranging from screening, nosebleed management, AVM treatment and more.[1]

Spreading Awareness at Medical Conferences

Cure HHT was an exhibitor at the American Society of Hematology meeting in San Diego in early December, the world's largest gathering of hematologists. We are committed to growing the medical community focusing on this disease!

Upcoming Conferences & Events!

Regional Conference Coming to Denver in March

Join us for our upcoming HHT Regional Patient Conference to connect with experts, learn about the latest treatments, and meet others in the HHT community **in Denver (Aurora), Colorado, March 28 – 29, 2025.**

This event will include a kids program. Learn more & register here: **curehht.org/denver-regional-conf-2025**.

The Future of HHT Needs You Join Our Legislative Action Network

As an individual impacted by HHT, you hold an incredible amount of power in your voice and unique story. While we work everyday to create pathways for change, federal funding is never guaranteed or promised every year. **We need your help to help drive the expansion of federal funding for our Centers of Excellence** – and there are many ways to get involved! Whether joining us in person in DC for future Hill Days, writing to your local officials, hosting meetings, coordinating volunteers locally and more.

If you're interested in using your voice to help create a brighter future today, we encourage you to raise your hand to join our Legislative Action Network by emailing **sparkchange@curehht.org**.

Upcoming National Conference: Dallas 2025

Mark your calendar for our next National HHT Conference in **Dallas, Texas in Fall of 2025**. There's no better way to stay updated on the latest in HHT treatment, care and research than by attending our conferences. Stay tuned for more.

[1] *[This activity is 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 $5,700,145 with 0% 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. For more information, please visit HRSA.gov.]*

Advance HHT Research: Donate Tissue Samples to our New BioBank

cure hht BIOBANK

We are excited to announce the launch of our centralized biobank dedicated to advancing research in HHT!

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. Researchers use tissue to look at the genetic changes that cause AVMs to occur, grow, persist, rupture, or stabilize. They also analyze the proteins and structural components between normal vessels and vascular lesions.

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.

Your participation is voluntary but could make a significant difference in the lives of those living with HHT. If you are interested in learning more about how you can contribute to this important cause, please contact Cassi Friday at **research@curehht.org** or visit **curehht.org/research/tools-education/donate-tissue**.

Participate in our Global Patient Registry

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.

Consider participating today: **curehht.org/hht-connect-registry**.

Share your HHT journey and help advance HHT research and care.

New Observational Study Alert! Help shape the future of HHT research and earn up to $1,300.

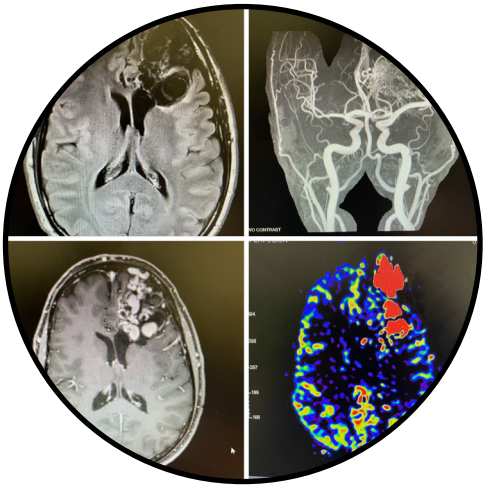
We invite you to join a new study dedicated to understanding the full picture of life with HHT. This fully remote, observational study requires no treatments or travel—just an opportunity to share your experience living with HHT.

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**.

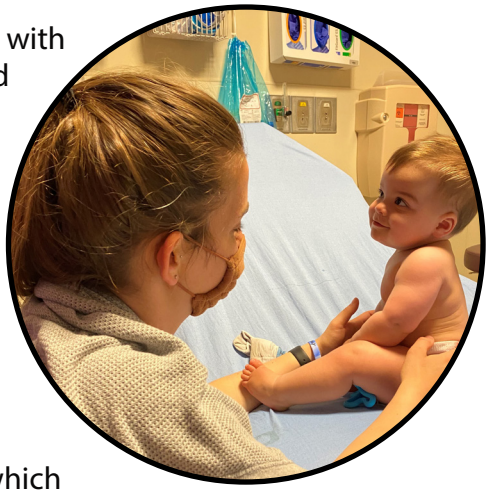
[The HHT Continuing Education Program is fully supported by the Health Resources and Services Administration (HRSA) of the U.S. Department of Health and Human Services (HHS) as part of an award totaling $5,700,145 with 0% financed with non-governmental sources. The contents are those of the author(s) and do not necessarily represent the official views of, nor an endorsement, by HRSA, HHS, or the U.S. Government. For more information, please visit HRSA.gov.

New Research: Importance of Screening Children for Brain VMs



In collaboration with Cure HHT, a new medical paper has been published that emphasizes the importance of screening children with HHT immediately for brain VMs. Some providers still believe screening before symptoms is unnecessary but this paper ("2024 Beslow et al. BAVM Screening Guidelines") counters those arguments.

What You Should Know: Don't Wait to Screen!



- Screening for brain VMs is recommended for children with HHT at the time of diagnosis using contrast-enhanced MRI. In other words, don't wait to screen!
- Early detection allows for proactive treatment, potentially preventing life-threatening hemorrhages.
- Current guidelines recommend this screening despite some practitioners' reservations due to the potential risks of sedation, contrast administration, and increased anxiety.
- The "watch and wait" approach is considered risky because it delays diagnosis until symptoms appear, which may be too late and lead to a preventable catastrophic event.

Marianne S. Clancy

Marianne S. Clancy, MPA
Executive Director, Cure HHT

As Hopeful a Time as There has Ever Been

I wish that everyone had the chance to be with us at our 15th International HHT Scientific Meeting, as the work displayed was a resounding reminder of how far we've come and how much hope is ahead. Our work over the years to better understand the underlying mechanisms of this disease has reached a place that is allowing us to develop treatments at the biological level. Just a few years ago, there were no HHT clinical trials and no interest from biotech and pharma. Today, our conference had an entire day dedicated to clinical trial presentations. Diagonal Therapeutics shared more about their antibody that can reverse disease, and dozens of other industry representatives were in the audience. It's all thanks to you – this community, and your support of our work. I'm more hopeful than I've ever been. We can end suffering from HHT this generation.

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