

Edition:
Summer
2022

Cure HHT

newsletter

News & Information for the HHT Community

A Time of Transformation

This is an exciting time for the HHT community. As we enter June Awareness month, the staff here at Cure HHT is abuzz. So much advancement has transpired over the last several months, so many milestones crossed, so much progress feels just within our reach. The newly-formed Cure HHT Research Network (CHRN) held its first conference this spring and has the potential to lead to groundbreaking advancements in treatments and research; after more than 15 years of effort, federal funding has finally been secured for our U.S. Centers of Excellence; promising clinical trials are underway; new centers are opening with others launching in the near future, making specialized HHT care more and more accessible to our entire community; and a host of research studies are in progress to unlock better knowledge and future treatments.

It is truly a transformative time. But now is not the time to let up. Critical work remains. And perhaps more urgently than ever before, we must keep the momentum going in order to turn these long-awaited advancements into a launching pad for true success.

Throughout the following pages, we welcome you to learn more about all the pivotal happenings that have the potential to transform and improve how we find, treat, and – one day – cure HHT. Together, with you, our incredibly supportive and generous community, we can create meaningful progress.

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Message from Marianne

T rue Transformation Is Within Our Reach –
But We Must Keep Pushing

Pausing isn't in my nature. Those that know me know this quite well. But given all the progress we have had in the last few months, I've been encouraged by many to take a brief second to reflect on how far we've come.

I've been part of this organization for nearly 30 years, and this year has been filled with more "pinch me" moments than I've ever experienced. From the 1st Cure HHT Research Network convening, and looking out at the crowd to see the smartest HHT minds in the world being

harnessed and focused in a way we could never have previously imagined, to unlocking federal funding after years of effort, to promising clinical trials underway and ahead... true transformation is just within our reach.

From where we were to where we are is simply remarkable. The future is undeniably bright. But enough reflecting. Because now more than ever we must keep pushing. We must harness this momentum to create even bigger wins, even faster advancements, and even more breakthroughs.

With our generous community at our side, I can't wait to continue this journey with you all. There is reason for optimism on the horizon. But it will take all of us to move these transformations forward.

Yours in good health,

Marianne S. Clancy, RDH, MPA
Executive Director, Cure HHT

***How do you want
to contribute toward creating
an HHT-free future?***

Email me any time at
marianne.clancy@curehht.org

Welcome Riley, Todd, Courtney and Gary!

With all the exciting possibilities just on the horizon, we are excited to share the latest additions to our team at Cure HHT – each of whom brings a unique skill set that will help us move forward in our mission to find, treat, and cure HHT.



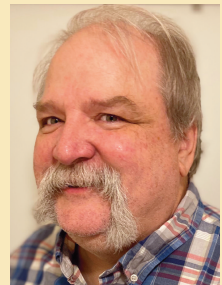
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CURE HHT NEWSLETTER

Marianne Clancy, RDH, MPA

Editor

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Marianne Clancy, RDH, MPA

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Barrow Named HHT Center of Excellence



Minding What Matters Most™

Cure HHT is excited to share that the **Barrow Neurological Institute** in Arizona has been established as the newest **HHT Center of Excellence**. This marks another exciting milestone in our quest to make specialized and multi-disciplinary HHT care more accessible to more of our patients around the country.

*"Barrow Neurological Institute has long been the mecca for brain AVMs," said **Michael T. Lawton, MD, president and CEO of Barrow**. "This is the place where the Spetzler-Martin grading system for AVMs was developed, where we have been refining that system with Lawton-Young supplementary grading, where management strategies like staged embolization and resection were popularized, and where we continue to be one of, if not the highest volume centers for AVM surgery—operating on more than 50 AVMs per year. It's fitting that we are now the HHT Center of Excellence in the region. We look forward to seeing patients with this diagnosis and delivering the best care to them."*



To learn more about Barrow's HHT program and team, visit www.barrowneuro.org. And be sure to stay tuned for more news in the near future about new HHT Centers of Excellence, as several new centers across the country are in the process of being formed.

Creating a Roadmap for the Future



Made possible through our **Chan Zuckerberg Initiative** grant, the **1st Cure HHT Research Network (CHRN) convening** took place in Boston in March, and there is real reason to be optimistic for the future of HHT treatment because of this patient-driven network.



With survey data from nearly 1,500 patients in hand and following hours of virtual working sessions well before arriving, the conference brought together patient advocates and 75 of the world's leading HHT clinicians, researchers, and scientists. The goal? Develop a roadmap prioritizing HHT research for the next 3-5 years based on what patients told us matters most to them. The result of this network is the type of focus and cross-functional collaboration we could have only dreamed about just a few years ago. Patients helped us identify the priorities, recommendations are in place to address the biggest research gaps, and strategies are being formulated to bring it all to life.



Cure HHT Executive Director Marianne Clancy called the conference “the most transformational” event she has ever attended in her near 30-years working for the foundation.

For more, you can read a letter from Marianne at: www.curehht.org/chnr-creating-a-roadmap-for-the-future/

Stay tuned, as we will have more to share about the CHRN and roadmap in the near future.

Jane Ribicoff Silk

– A Tribute



"Our family suffered a tragic loss with the death of our beloved mother, grandmother and my dear wife, Jane Silk. Her commitment to aid in the development of HHT Centers of Excellence (CoEs) was fueled by her loving nature and by memories of her grandmother's and mother's HHT driven transfusion dependency, and her deep devotion to her daughter and granddaughter, whose personal HHT stories have yet to fully unfold. If she were alive today, she would be pleased to know that U.S. HHT CoEs have recently secured significant, initial federal funding. We in her family are so grateful to see the organization continue to grow yet saddened that Jane could not be here to share in our hope for the future."

– Stephen R. Silk, PhD

My Tribute to Jane Ribicoff Silk

By Marianne S. Clancy

My heart was broken when I lost my dear friend and HHT Champion, Jane Silk, in November 2021, from complications associated with HHT.

I first met Jane in 1999 when I became President of HHT Foundation International (now Cure HHT) and she was a Board member. Jane had a quick wit, sharp mind, and was focused on the future — a future that advanced new treatments and an eventual cure for the disease that caused great suffering for her mother and grandmother.

When I became Executive Director in 2001, Jane became President of the Board. We were focused on expanding this young organization and to fund research. In 2003, together with a fellow Board member, Diana Meiches, and Dr. Bob White, we began fundraising across the country.... and....raise money we did! By 2005, we raised \$1.8 million for HHT research from generous families invested in seeing a brighter future.

We began our legislative advocacy in 2005. Jane did not want to be in the spotlight. Because of her family's political background, she and I were able to meet very prominent Senators, Congressmen/women and others in the political world. She was humble and knew what needed to be accomplished. Jane got right to work! Her strong organization skills along with writing and editing skills were invaluable through the years as we were building Cure HHT into a strong and viable organization. She was instrumental in our successful efforts in conference fundraising and was generous to the Foundation. Jane extended her outreach to friends who also contributed generously to Cure HHT. Her efforts advanced the research that Cure HHT has sponsored through the years.

Through the years, Jane took time out to email and phone the Cure HHT staff to compliment their work, whether it be the newsletter, e-mail communications, or conference programming and execution.

In March 2022, we realized our dream of federal funding for our HHT Centers of Excellence. Jane and I worked continuously for 17 years along with so many others to reach this achievement. As recently as last fall, we met with Senate and House members to advocate strongly for funding. I only wish she would have lived a few more months to see this accomplishment....This is her legacy....I miss my friend and think of her so often....what I miss most is our laughter....



We Did It: Federal Funding Secured

It is with great joy that we share the news that our funding request for HHT Centers of Excellence was included in the final federal Appropriations spending bill signed into law by President Biden in March. The new “National HHT Diagnosis and Treatment Initiative” provides \$2 million to our existing U.S. CoEs to help expand staffing and improve infrastructure, as well aid in establishing new CoEs.

Up until now, our 24 CoEs in the U.S. have had no access to federal funding despite HHT being the second most common genetic bleeding disorder. This is a journey that began over 15 years ago, and is the culmination of countless trips to Capitol Hill by Cure HHT staff, CoE directors, patient advocates, and more.

None of this would be possible without all of you, our powerful HHT community. Our thanks go out to all of those who came to Washington with us or met in regional offices with your local Representatives. So many of you shared your stories through personal letters with your leaders to emphasize why this cause truly deserves to be funded. We asked for your help and you delivered!

Our patients and families truly are a catalyst for change. Your time, efforts and generous donations give us the resources we need to continue our work in making a better life for those who suffer from HHT. Again, this is a transformational win for our community. But also again, work remains to ensure this is only the beginning!



HHT patients, families, volunteers, staff and Board came to Washington, D.C. several times to advocate for federal funding.

Marianne Clancy: *So Many To Thank*

Years of effort, time and investment have led to this finally becoming a reality. Our late Board of Directors' president, Jane Ribicoff Silk and I began on this journey in 2005.

With our legislative consultant Mark Vieth at our side, we made countless trips to Capitol Hill. I must acknowledge:

- ◆ Dr. Jeff Pollak and Kate Henderson at Yale University School of Medicine
- ◆ Dr. Vivek Iyer at Mayo Clinic
- ◆ Dr. Raj Kasthuri at University of North Carolina
- ◆ Dr. Murali Chakinala and Dr. Andy White at Washington University
- ◆ Dr. Kevin Whitehead and Jamie McDonald at University of Utah
- ◆ Dr. Jim Gossage at Augusta University
- ◆ Dr. Bob White
- ◆ All our patients who made calls and helped advocate
- ◆ Our donors – this is not possible without you
- ◆ Our mighty staff at Cure HHT!



Capitol Hill Visit by Cure HHT Staff

Visiting Representatives on Capitol Hill



NOW, HELP US CONTINUE TO FUND OUR ADVOCACY!

Advocacy works! However, the federal funding we just leveraged through our efforts will support our U.S. CoEs, not Cure HHT. It takes time (17 years!), money and staff to continue our efforts for recognition and funding with the federal government, and with the agencies that provide research grants.

You can help! We rely on your generous tax-deductible contributions to continue our vital work. Please consider a special gift today to support our advocacy efforts! *Thank you in advance for your generosity!*

When it Comes to HHT, Knowledge is Power

With as many as 90% of people with HHT undiagnosed, heightening awareness among the medical and scientific community is key – as is continuing to generate interest for young doctors and researchers to want to focus on this disease.



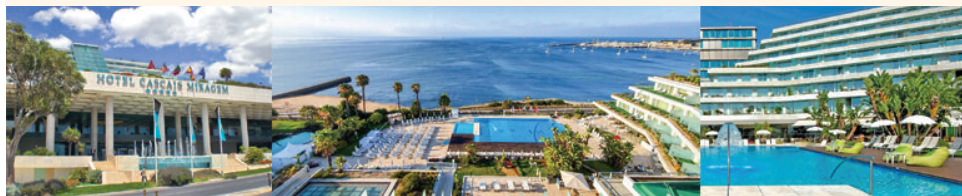
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14TH HHT INTERNATIONAL SCIENTIFIC CONFERENCE

September 29, 2022 – October 2, 2022

SAVE THE DATES!

Hotel Cascais Miragem Health & Spa
Estoril, Cascais, Portugal



- Abstract submission portal is now open
- Registration will open June 1, 2022

Bringing Clinicians and Scientists Together

Every two years, clinicians and scientists from around the world gather at our Scientific Conference to discuss the latest advances in HHT research. This important meeting leads to expanding scientific collaborations that accelerate progress in HHT research, treatment, and extending patient access to expert care. This year's meeting will take place from **Sept. 29 – Oct. 2**. Our generous community can make donations to help young investigators attend, creating a brighter tomorrow for HHT treatment.

Please visit www.curehht.org/ways-to-donate

Cure HHT International Expert Case Conference

In response to requests from our medical and scientific community for more collaboration, Cure HHT launched the **International Expert Case Conference** last year. With the ultimate goal to create dialog among HHT CoE personnel to improve patient care, here is the upcoming topic schedule.

- **May 31 @ 5pm EST – Genetics** hosted by University of Pennsylvania HHT Center of Excellence
- **July 20 @ 5pm EST – Pediatrics** hosted by Cincinnati HHT Center of Excellence
- **September (date TBD) – Stroke** hosted by Barrow Neurological Institute HHT Center of Excellence
- **November (TBD) Hematology** hosted by Froedtert & MCW HHT Center of Excellence

Let The World Hear Us

June Awareness Month 2022

Once again, it's time for us to do what we do best as a community—spread Awareness about HHT! June Awareness Month 2022 is here, and we have BIG plans!

Here are a few simple ways to get involved and **#LetTheWorldHearUs!**

1. **Share your story** — We can't emphasize enough how important your HHT story means. Each of you have a personal connection to this disease. Sharing your experiences, the good and the bad, can make all the difference in the world! Post your story to your favorite social media channels and use the hashtag *#LetTheWorldHearUs* to make an impact around the globe.
2. **Snap a Pic** — Purchase one of our inflatable globes from the Cure HHT Store between now and **June Awareness Day (23rd)** and snap a picture with your globe, then post to social media and use the hashtag *#LetTheWorldHearUs*. We want to flood social media with our HHT Community pictures and show the world that we will be heard! www.curehht.org/store
3. **Join Strides for Strength** — Rally your family and friends together and participate in our global virtual event. This year you can walk, run or ride to show your support and spread awareness in your corner of the world. Get out there! www.curehht.org/strides-for-strength
4. **Donate** — Help us raise \$50,000 by June 30th (all donations will be DOUBLED thanks to a generous donor) to support lasting national and global collaborations among the HHT patient, medical, and scientific communities – aimed at improving the lives of HHT patients everywhere. Here are a few ways we are putting your donations to work:
 - *International Science Conference* – a worldwide convening of the top HHT minds!
 - *Travel grants* for Young Investigators, residents, and fellows to attend professional meetings – extending opportunities to the next generation of HHT physicians and scientists who may someday cure this disease!
 - *Education of patients and providers* through the HHT Academy – continuing our efforts to provide relevant educational opportunities to as many as possible.

Make your donation today at <http://www.curehht.org/awareness>

Join us throughout June as we rally our global community. Together, we can bring attention to HHT — a disease that impacts 1.4 million people yet 90% remain undiagnosed. Exciting progress is happening, and we are chipping away at that number but there is still important work to be done. ***#LetTheWorldHearUs today!***

Children from the Young and Gorman families are ready for June Awareness Month, and want the world to hear them!



Empowering the Patient Voice

We ensure the patient voice is at the center of the work being done to inform the future of treatment. Learn more about how the patient voice has played a role, and current opportunities for your involvement.

Completed Studies

Assessing How HHT Affects Patients Quality of Life

Cure HHT funded a research grant at **The HHT CoE at Johns Hopkins School of Medicine** to help develop a standardized quality-of-life metric tool tailored to the global illness experience of those with HHT. **Thank you** to all those who completed the survey to help us better understand how the disease affects patients' physical, emotional, and mental health.



Deepening Understanding of Telangiectasia Care

Researchers at the **Augusta University HHT Center of Excellence** recently conducted a research study to learn more about the care that HHT patients receive for cutaneous telangiectasia. Thanks to our determined community, Dr. James Gossage and team received nearly 800 responses – well exceeding their target for the study.



Studies Still in Progress

Brain AVM Study – *Participants Still Needed!*

HHT Centers of Excellence are still actively recruiting patients to participate in an NIH funded study, entitled “**Cerebral Hemorrhage Risk in Hereditary Hemorrhagic Telangiectasia (HHT).**” The goal of this research is to determine what genetic and clinical factors signal high risk for hemorrhage from brain AVMs.

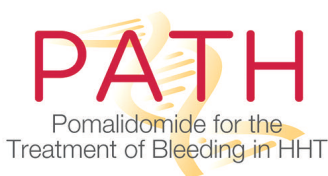
HHT patients with a brain AVM, whether or not it's been treated, should contact Leslie Perry, RN by email (research@curehht.org) or 410-357-9932 to determine eligibility.



Recruiting for PATH Study

Cure HHT is helping to recruit adult HHT patients with moderate to severe nosebleeds who have anemia or require iron infusions or blood transfusions to participate in **PATH**, a research study exploring the use of an oral medication called pomalidomide.

To learn more, visit www.path-hht.org



FREE Genetic Testing for Rare Diseases

The **Rare Genomes Project** is a *free* and fully remote research project that provides genomic sequencing. The goal is to provide you with the genetic diagnosis you're looking for and discover the genes underlying rare diseases like HHT at the same time.

Learn more at: www.curehht.org/rare-genomes-project/



Strides for Strength – *Lace Up Those Shoes!*



June 4th - 19th

Our 3rd annual Global Virtual Walk for a Cure, Strides for Strength, is back with more ways to get involved this **June Awareness Month**. Now, you can walk, run or RIDE—all in the spirit of raising awareness.

Join the global HHT Community and help us raise \$75,000 for a cure. For the first time ever, we are pleased to announce that Strides for Strength donations will be matched—thanks to a generous donor—up to \$25,000 making your donations get double the traction!

Register as an individual, form a team, or join an existing team. Register the whole family, including your furry friends, and spread awareness together in your own hometown. **Registration is now open!**
www.curehht.org/strides-for-strength



Cure HHT's 1st Virtual Patient/Physician Conference

Our first virtual patient and physician conference, HHT Academy, was a not-to-be missed event! Hosted this spring, the nine-day learning and information extravaganza featured a mix of patient and physician focused tracks, meet-and-greets, targeted Q&As with HHT experts and more!
Thank you to all our sponsors for making it happen:



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Providing Resources To Support Our Community

One way we support our patient community is by working to provide meaningful resources to help patients and their families to better manage, understand, and advocate for their treatment.

– Digital Version
Now Available!



Download the Digital Guide Today!

As a reminder, our comprehensive **Guide to Managing Your HHT** is now available as a digital download. This 48-page booklet contains ten fact sheets summarizing the latest International HHT Guidelines. Paper copies are still available and ready for shipping.

Get your copy at: www.curehht.org/managingyourhht_booklet

Scientific Newsletter

Did you know that twice a year, Cure HHT collaborates with its global research and medical advisory board to publish **HHT Frontlines** – a scientific newsletter aimed at centralizing and summarizing the most relevant HHT studies with the scientific and medical community?

While the newsletter focuses on topics most relevant to the medical community, our **Resource Library** (www.curehht.org/resource-library/) serves as a comprehensive resource for our entire community where you can find a wide range of content on the latest HHT information, research, fact sheets, and more!





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