



Full Speed Ahead in 2022

With spring rapidly approaching, we are energized with the promise of longer, brighter days as we embark on our most innovative medical advances and physician education initiatives in our organization’s thirty-year history. Building on our past successes, we continue to create opportunities and life-changing resources for generations of families to live their best lives.

When we reflect on our accomplishments in the past year, we recognize just how powerful and far-reaching our efforts have travelled. By building global research partnerships, investing in basic science to progress to translational research, and creating a patient research network through the Chan Zuckerberg Initiative (“CZI”), 2021 took the HHT Community on a journey that earlier generations only dreamed. Now these dreams have blossomed into a reality where patients and their families can confidentially maintain hope for a healthier future.

Throughout the following pages, we welcome you to explore our commitment to transforming patient care with the planned opening of new HHT Centers of Excellence; pivotal research that will introduce effective treatments and our first FDA approved drug; trailblazing medical education to increase awareness and substantially decrease the lag in diagnosis; and building partnerships and patient engagement through our global patient research network and registry. We remain on the right track in our three-pronged mission of finding, treating, and curing HHT. This journey with you, our most supportive community, remains full speed ahead.

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Message from Marianne

I would like to tell you a story.

We are excited to share the great news of a major research grant to advance a drug therapy for HHT treatment! We have been working over several years to reach this important milestone: it is through sheer determination and investment by our Board that a Phase II-III multi-center clinical trial for this therapy will launch in mid-2022! If all goes according to plan, upon completion of a successful trial, we will seek FDA Registration as our first approved drug therapy for HHT treatment. Registration would ensure insurance and Medicare coverage for this drug.

You may recall, an initial small scale trial was funded and started with Glaxo Smith Kline (GSK) as our

partner. A repurposed drug, Pazopanib, was created in **pill** form and 7 of the planned 30 patients were recruited and completed the trial. Positive results were reported in nosebleed reduction and a return to normal hemoglobin with no adverse effects. In some patients, the drug helped reduce heart failure due to liver avms, a major added benefit. Unfortunately, the trial ended when GSK sold this drug with its cancer drug portfolio to another company. We advocated for over a year for the new company to continue the trial, but they chose not to do so.

We were disappointed, but not deterred. With significant volunteer help from a member of our community with pharma experience, and a few of our center physicians who continued to prescribe this therapy for patients, we were able to advance our cause.

With help from you and our donors, over the last 3 years, we were able to accomplish the following:

- Obtained the generic form of Pazopanib, found a manufacturer, and collaborated with the University of Utah to conduct a safety and stability study which produced positive results. Our Board agreed to invest in this research with hopes of a future clinical trial.
- Sought and received Orphan Drug Breakthrough Designation from the FDA! This is not an easy feat. It was the voice of the patients and the strong signals from the GSK study that helped our application.
- Submitted grant applications to the federal government to fund this study at HHT Centers of Excellence; provided Cure HHT staff resources to assist in drafting the Investigational New Drug application to the FDA and the FDA Breakthrough designation as well as two grant applications to fund the multi-center clinical trial.

Our FDA grant was approved! Bells were ringing in our office and our excitement could not be contained! A study protocol for reducing bleeding using pazopanib in pill form was created, and 12 HHT Centers of Excellence will begin recruitment this year of 70 geographically dispersed patients. The trial will end in 2024. To say we are elated is an understatement.

Cure HHT continues to be the “Little Engine That Could” and we are on our way to advancing and owning therapies critical for patient care. Your continued support will help further these initiatives for us all. Thank You!

Yours in good health,

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



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youtube.com/HHTFoundation



instagram.com/cure_hht



<https://curehht.org/blog>

Stay current on HHT clinical trials, education programs and community events. Sign up to receive electronic announcements by registering at curehht.org or send us an email at hhtinfo@curehht.org

CURE HHT NEWSLETTER

Marianne Clancy, RDH, MPA
Editor

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CZI Grant Extended

We are thrilled to announce that our grant in the **Chan Zuckerberg Initiative – Rare As One** will be extended for another year!

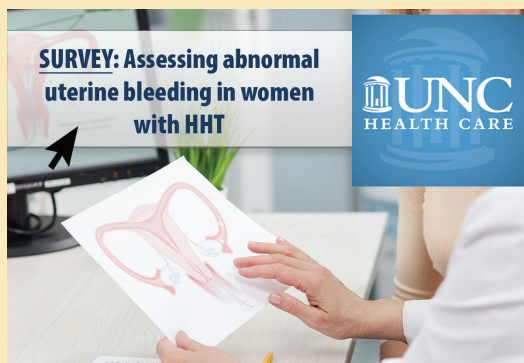
It's hard to believe it has been 2 years since we were first selected as one of only thirty rare disease organizations to be a part of CZI – Rare as One. Over the past 24 months, we have soaked up all the knowledge, discussion, learning, and resources CZI had to offer as part of our grant—all with a singular focus, to provide MORE for our global patient community with a particular emphasis on RESEARCH. As an organization, we have put those resources to work and have made leaps and bounds to expand our international reach, convene our community towards patient driven research, and identify researcher-patient common priorities.

We are proud of the launch of our first research network which many of you have already participated. Having our patients drive research is something we have envisioned for years. Now, with the help of the Chan Zuckerberg Initiative, we have been able to see the Cure HHT research network come alive. We look forward to continued collaboration with CZI and our patients as we elevate patient-driven research to the next level. To learn more about our research network, visit: www.curehhtresearchnetwork.org

Women with HHT Pave the Way for Research

For years, targeting women's issues in relation to HHT has long been a neglected area in HHT research. Last month, thanks to the leadership of Dr. Raj Kasthuri, Director, HHT Center of Excellence at the University of North Carolina, and his team, an important research survey was populated throughout the HHT Community involving the prevalence and severity of heavy menstrual bleeding in women with HHT.

Thanks to our fierce and determined female community, Dr. Kasthuri and his team reached their goal of 500+ participants, enabling them to gather needed information to determine future clinical research surrounding the prevalence and severity of abnormal uterine bleeding in women with HHT. Study results will be set for later publication and presentation at this fall's HHT International Scientific Conference. This would not have been made possible without the time and dedication of the UNC Team and our incredible HHT female community.



Our Newest Resources for Managing Your HHT



We are pleased to announce that 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.

Each fact sheet contains:

- ◆ Signs and Symptoms
- ◆ Important Facts to Remember
- ◆ How HHT is Diagnosed
- ◆ Treatment
- ◆ Discussion Points for You and Your Physician

The fact sheets have corresponding “My HHT Care Checklists” which are a handy resource for managing screenings and treatment options outlined directly from the published International HHT Guidelines. It's a great way to share information with your physicians, particularly those who are not affiliated with an HHT Center of Excellence.

Download your copy today at:
www.curehht.org/managingyourhht_booklet

Prefer a paper copy to make notes or share? Your first copy remains complimentary. Just indicate your choice of a paper copy on the link above or complete our Join Us form www.curehht.org/joinus to receive your complimentary copy and all the latest buzz from the foundation. Additional copies of the booklet are available through the Cure HHT store www.curehht.org/store



THANK YOU!
WE EXCEEDED OUR YEAR END GOAL!

*Let's start with **WOW** and leave it there for a minute!*

Your incredible generosity to our #OnTheRightTrack Year-End Matching Gift campaign, accomplished just that— put us on the right track for the coming year. Thanks to each of you, we exceeded our 2021 Matching Gift Campaign goal, which has been doubled with an additional \$300,000 in matching funds from our group of generous donors – *our largest match ever!*

Education of Health Care Providers is a Major Challenge!

Even as we celebrate our 30th anniversary with remarkable breakthroughs and major advances, we need to increase our efforts to educate the medical community about HHT. HHT is still unknown, unrecognized and misdiagnosed - sometimes with disastrous consequences for the approximate 90% who are unknowingly living with HHT.

Your donations give us the resources needed to tackle an **aggressive educational push**—one that has been long overdue. We feel a sense of renewed excitement and energy to push forward every day this year as we continue to work hard for a community that has never let us down.

Thank you again for your trust and support as we continue our challenging work in making a better life for those that suffer from HHT. We are fortunate to have such a strong bond with our community and will remain #OnTheRightTrack for each and every one of you!

Sincere best wishes,

Marianne S. Clancy

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



Syrus (age 4) - suffers from brain AVMs

2022 Physician Education Opportunities

Some of these opportunities are already being implemented, and some are on our “wish list” – your support will make them possible!

American Society of Pediatric Hematology and Oncology (“ASPHO”) Annual Meeting – Attendance and presentation at this meeting will allow our Executive Director, Marianne Clancy, the opportunity to connect with pediatric hematologists all across the country.

American College of Emergency Physicians (“ACEP”) – ACEP has a membership of 38,000+ emergency room personnel. Our goal is to provide a webinar or live presentation to physicians who are on the front lines seeing patients with massive nosebleeds and never make the connection to HHT.

National School Nurses Association – We have the ability to present an online education event with a potential audience of 85,000+ nationwide, opening the door for HHT awareness and knowledge across the nation in your very own neighborhood!

5th Annual Aspen Conference on Pediatric Cerebrovascular Disease and Stroke – Hosted by the International Society for the Study of Vascular Anomalies - an amazing opportunity to educate pediatricians, pediatric neurosurgeons and other specialists.

Centers for Disease Control and Prevention (“CDC”) – Feature spotlight in June for HHT Awareness Month – This first ever spotlight would have the potential to reach 50,000+ healthcare providers highlighting the varying signs and symptoms of HHT between adults and children.

International Pulmonary Hypertension Conference and Scientific Sessions – An informational booth hosted by our Chief Medical Officer, Dr. Scott Olitsky explaining the connection between HHT and PH. Many PH patients who suffer from HHT remain undiagnosed.

National Hemophilia Foundation Bleeding Disorders Conference – A presentation by one of HHT’s top hematologists and HHT Center Director at UNC Chapel Hill, Dr. Raj Kasthuri. Dr. Kasthuri would provide a wealth of knowledge to thousands of hematologists, which could lead to game-changing management of HHT related anemia.

Cure HHT International Grand Rounds – Cure HHT is dedicated to expanding our international reach in 2022 and helping those who do not have access to quality care.

Cure HHT International Scientific Conference – Cure HHT will host our biannual scientific conference in September, 2022 in Portugal, bringing the greatest HHT minds together—clinicians, scientists, and researchers to collaborate, discuss, inform, and teach everything HHT.

***Brand New* – Cure HHT Academy** – Over 10 days, educational tracks will be built specifically for physicians. As this will be a virtual event, physicians from around the world will have this unique opportunity to participate.

Teach a patient about HHT, save a life.
Teach a doctor about HHT, save a hundred lives!

Your donations make all this and more possible! None of our physician education efforts are grant funded. Our small but dedicated team relies entirely on your generosity to keep our engine at full speed. With your support, we have proven time and again that Cure HHT will always reach our destination – we are on the right track!

NOTICE OF CLINICAL TRIAL

POMALIDOMIDE FOR THE TREATMENT OF BLEEDING IN HHT (PATH)

We are recruiting 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**.

Clinical research is essential in our fight to cure HHT. Your participation has a huge impact on the diagnosis and treatment of HHT. Diseases can only be cured when scientists understand them. This understanding, which comes from basic research, leads to new drugs and treatments. *Without you, the advancement of a cure for HHT is impossible!*

STUDY GOAL

To determine the efficacy of pomalidomide compared to placebo for the reduction in severity of nosebleeds after 24 weeks of treatment.

STUDY DURATION

8-month study in which patients might receive either pomalidomide or a matching placebo (sugar pill) in addition to their usual care.

Recruitment will take place at up to 15 HHT research centers across the United States. Learn more and review eligibility criteria by visiting the following:

<https://path-hht.org>

<https://clinicaltrials.gov/ct2/show/NCT03910244>

<https://CureHHT.org/research/participate-in-research/clinical-trials>

*PATH is funded by a grant from the National Heart, Lung and Blood Institute (NHLBI), is led by researchers at the Cleveland Clinic and RTI International.

**Pomalidomide is an FDA-approved drug for the treatment of some cancers and is manufactured by Celgene.

Brain AVM Study Continues – Participants Needed

Cure HHT, along with HHT Centers of Excellence across North America are actively recruiting HHT patients worldwide to participate in the next round of 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 its been treated, should contact Leslie Perry, RN by email (research@curehht.org) or 410-357-9932 to determine eligibility.

NO TRAVEL required, information gathering only, one hour of your time.



Duke University Heads Live Tissue Research for HHT

Duke University, through a separate BVMC project, is collecting **vascular malformations (VMs)** associated with HHT to search for somatic mutations, possibly occurring in different genes than the ones already identified.

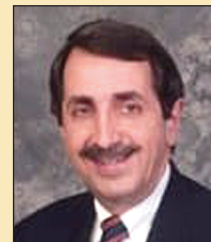
All HHT patients undergoing surgery to remove an AVM from any organ (i.e., liver, lung, brain, skin, etc.), are encouraged to consider donating tissue.

You do not need to have a brain AVM to participate in this study project.

Contact Leslie Perry, RN @ research@curehht.org or (410) 357-9932 for additional information.

BOARD ANNOUNCEMENTS

As the Board of Directors' door closes for these board members, we would like to acknowledge their steadfast dedication to our mission and the tremendous impact they each made to the organization. Our biggest thanks to each of you.



CHARLES INGARDIA, MD



GARY MCKEE, MD

Welcome Howard, Savannah and Scott!

Introducing our newest board members, each bringing a unique skill set and knowledge to the organization. We are excited to collaborate with each of them as we journey forward together to find, treat and cure HHT!



SCOTT BOATWRIGHT



HOWARD LICHTIG



SAVANAH SCHOTT, MBA



This study is funded by NHLBI: Pomalidomide for Bleeding in Patients with Hereditary Hemorrhagic Telangiectasia (HHT)

Indiana HTC-HHT Treatment Center Make Their Mark in Indianapolis



Since IHTC came on board in 2021 as an HHT Treatment Center, they have been a force in diagnosing, treating, and managing HHT patients throughout Indiana and beyond. Located within one of the nation's largest hemophilia treatment centers (and Indiana's only federally designated HTC), IHTC provides patients access to its onsite multidisciplinary staff including: physical therapy, dietary, dental hygiene, social work, and academic/career counseling. With onsite genetic counseling and testing, including screening for at-risk relatives along with access to referral and care coordination with other specialists such as cardiology, pulmonology, neurology, gastroenterology, and interventional radiology, IHTC is an invaluable asset to the HHT Community.

Meet IHTC's Dedicated HHT Care Team:



Anthony Betabdal, MD



Brandon Hardesty, MD



Magdalena Lewandowska, MD, Director



Charles Nakar, ND



Angeli Rampersad



Shelby Gordon, RN,
HHT Clinic Coordinator



Meadow Heiman,
Genetic Counselor

To start your own HHT treatment journey with IHTC,
contact their HHT Center Coordinator, Shelby Gordon, today!
8:00 a.m. - 4:30 p.m., Monday through Friday

Indiana Hemophilia & Thrombosis Center
8326 Naab Road Indianapolis, IN 46260
Direct: 317.871.0000 Toll Free: 1.877.256.8837

Please visit IHTC online at www.ihtc.org/hht-clinic
to access the Patient Portal.

The Indiana Hemophilia and Thrombosis HHT Treatment Center
is a proud sponsor of our **2021 Strides for Strength Global Walk**.
We are extremely grateful for their support.

Coming Soon - Cure HHT's 1st Virtual Patient/Physician Conference



Conference Dates: April 29 - May 7, 2022

The Cure HHT Academy is pleased to present its
1st Virtual Patient/Physician Conference this spring.

The conference, a nine-day learning and information
gathering extravaganza, will feature:

- Patient and physician focused tracks – select any or all of the nine tracks and curate a program just for you
- Personal meet and greets with HHT physicians and scientists
- Exclusive swag upon registration
- Exciting clinical trial/research announcements – be the first to know!
- Daily giveaways
- Personalized Participation Certificates
- Targeted Q & A sessions with HHT experts and so much more

Don't miss this incredible opportunity to expand your HHT knowledge and connect personally with the HHT Community of patients and physicians.

Registration opens early April.

Strides for Strength – *Third Time's a Charm!*



Our 3rd Annual Global Virtual Walk for a Cure – Strides for Strength is headed your way again this **June Awareness Month**. Mark your calendar for this exciting event when the HHT Community from around the world bands together to walk for a cure.

Save the date – June 4th – June 19th.

More details coming soon. www.curehht.org/strides-for-strength

TDC Leaps to cure HHT



Maria Mahoney & daughter, Easton

The competition team at **The Dance Connection (TDC)** in Unionville, CT leaped high to raise awareness for HHT. The event was organized to show love and support to studio owner, Maria Mahoney, who was diagnosed with HHT in 1988 by the Yale New Haven Hospitals HHT Center of Excellence. Maria's children were later diagnosed as well. Maria strives to do whatever she can to ensure her children have the best quality of life living with the disease. The team recognized Maria's dedication to raising HHT Awareness and wanted to contribute in an impactful way.

The **"TDC Leaps for Cure HHT"** team was excited to work together for a great cause. As a result of generous donations, the team goal of \$1,000 was far exceeded. Nearly \$2,500 was raised to further research. The team is looking forward to making this an annual event. Sending a high five to this amazing group of young women and girls for their tremendous efforts to LEAP for awareness and funds for HHT.





September 29, 2022 – October 2, 2022
Hotel Cascais Miragem Health & Spa
Estoril, Cascais, Portugal

If you no longer wish to receive this newsletter please
contact hhtinfo@curehht.org or 410-357-9932.