

Edition:
Spring
2021

Cure HHT newsletter

News & Information for the HHT Community

THIRTY YEARS STRONG

For three decades, **Cure HHT** has been the “A-Team” for the HHT Community—advancing Awareness, Advocacy, and Access to care. Year after year, those advances have secured Cure HHT’s status as the Cornerstone of the HHT Community—a perfect union of our past, present, and future. Today, we continue pledging our dedication to leveraging funding for research and advocating for the 1.4 million people worldwide who have come to rely on us in helping to find, treat and cure HHT. This has been our mission since our early beginnings and continues to motivate us into the future as the world’s leading organization in the fight against HHT.

As Cure HHT boldly moves into the next decade, we are excited for our newest and most promising initiatives to date. We want to share in that excitement with you—our growing and flourishing community of patients, caregivers, physicians, and scientists—who have helped us build from the ground up and gave us the strength to persevere for the last 30 years. Humbly we know, we would not be where we are today without each and every one of you!

Journey inside these twelve pages of news, research, resources, and events and witness the excitement of what’s to come. Become a part of something bigger than all of us—Hope! Every step we take with YOU is an opportunity to strengthen a promising future together as we embark on our most aggressive goals yet, never forgetting the universal goal for each of us—a Cure!



TABLE OF CONTENTS

- | | | | |
|------------|--|-----------|--|
| 2 | Message from Marianne | 8 | Cure HHT Research Network Update |
| 3 | New HHT-HTC Treatment Centers | 9 | Is HHT a Bleeding Disorder? |
| 4 | Our Best Resources for You! | 10 | Kids Making An Impact |
| 6-7 | Advocacy - How Your Gifts
Help Researchers Raise Millions | 11 | Strides for Strength is Back!
& June Awareness Month Calendar |



Message from Marianne

Thirty Years of Support, Access to Expert Care, Research and Advocacy

Cure HHT has been there for me since the beginning... When I received my diagnosis of HHT in 1994, I started to connect the dots of several events in my life. My sister died when I was 18 months old, and I lost my mother to HHT when I was only 21, not to mention years of unexplained nosebleeds, family members with strokes, hemorrhages, and anemia. Receiving my diagnosis was both empowering and terrifying; on one hand, I finally had some answers, but on the other hand, I had a thousand new questions. With three young children, receiving a genetic diagnosis had me worried now for my new family. Luckily, the HHT Foundation (formerly known) was there to support me.

At that time, the three-year-old organization consisted completely of volunteers and they took the time to listen to my concerns and arm me with the knowledge to advocate for myself. Even more importantly, they provided me the comfort of knowing that I was not alone.

I have been with the HHT Foundation (now Cure HHT) for 26 years: as a Board member, volunteer President, and the first Executive Director. I have watched the organization grow from volunteer-based to a paid staff of nine. I've watched them work tirelessly to open 27 Centers of Excellence to ensure that patients can access the expert HHT care that they need. I've been to almost every patient conference, 10 Scientific Conferences, and two Clinical Guidelines conferences. I've seen the organization take something small and leverage it to make a big impact; we've invested \$1.4M in small research grants, that resulted in \$44.8M of federal funding of HHT research. I've seen this organization work to earn so many exceptional achievements only furthering my excitement for all the future possibilities.

So, happy 30th anniversary Cure HHT. We have very special plans to note this important anniversary. You have been there for thousands of families like mine, and we are committed to advancing new treatments--several on the very near horizon and finally cure this disease. Our HHT Community deserves nothing less.

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

BOARD ANNOUNCEMENTS

We are excited to introduce the next generation of new Board members into our fold. Each with their own unique contributions, we look forward to their fresh perspectives, professional insight, and individual skill sets in leading Cure HHT into the future.



ANTHONY ANZELL, Ph.D.



GABRIELLA "Gabby" CHOI



BRANDON PROBST



CONNECT WITH US



facebook.com/hht.org



linkedin.com/company/curehht



[@curehht](https://twitter.com/curehht)



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

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

Marianne Clancy, RDH, MPA

Executive Director

Scott Olitsky, M.D., M.B.A.

Chief Medical Officer

Hans-Jürgen Mager, M.D., Ph.D.

*Chair, Global Research
and Medical Advisory Board*

Christopher Hughes, Ph.D.

*Chair, North American Science
and Medical Advisory Council*

BOARD OF DIRECTORS

Sara Palmer, Ph.D.

President

Ben Holt, Esquire

Vice President

Mark Topaz, Esquire

Treasurer

John Dunn

Secretary

Anthony Anzell, Ph.D.

Harry Chapman

Gabriella Choi

Charles Ingardia, M.D.

Jim Lapides

Marijo McCune

Gary McKee, M.D.

Scott Olitsky, M.D., M.B.A.

Brandon Probst

Dennis Sprecher, M.D.

Ann Trussell

Karen Silber Weissel, Ph.D.

Robert Berkman

Member Emeritus

Dennis Routledge

Member Emeritus

New HHT-HTC Treatment Centers

Cure HHT is excited to announce that the **Indiana Hemophilia & Thrombosis Center** and the **University of Michigan Hemophilia & Thrombosis Center**, both Hemophilia Treatment Centers (HTCs), are now operational as HHT-HTC Treatment Centers.



Thanks to a new program proposed by Cure HHT and funded by the Centers for Disease Control and Prevention ("CDC"), Cure HHT was able to collaborate with the American Thrombosis & Hemostasis Network ("ATHN") to train HTC staff, identify subspecialists necessary to provide HHT care, and establish a patient referral/treatment program for HHT patients at these exceptional HTCs located in **Indianapolis, Indiana** and **Ann Arbor, Michigan**. The main goal of these partnerships is to explore alternative ways to provide multi-disciplinary HHT care at established HTCs, which could have a tremendous impact on patient access to expert care. This is particularly true in states that do not currently have an HHT Center of Excellence.

Both HHT-HTCs are currently accepting new patients and welcome your inquiries. To learn more about these Centers, visit <https://directory.curehht.org/hht-centers> for center contact information and staffing.



"The Indiana Hemophilia & Thrombosis Center is excited to announce our partnership with Cure HHT as an HHT-HTC Treatment Center. This designation is truly an honor and reinforces our center's ongoing commitment to providing expert HHT care, allowing our patients to flourish."

– **Magdalena Lewandowska, MD**, Director,
Indiana HHT-HTC Treatment Center



"We at Michigan Medicine are excited to join the HHT community. At our Hemophilia Treatment Center (HTC) we are already working with our colleagues to treat a wide range of patients with HHT. We look forward to developing our program to become a full Center of Excellence in the near future."

– **Suman Sood, MD**, Director,
University of Michigan HHT-HTC Treatment Center

Our Best Resources – Available to You!

This past December, we launched our most comprehensive resource for managing your HHT. Ironically enough, that's exactly what we called it, **"A Comprehensive Guide to Managing your HHT."** This 48 page booklet contains ten Fact Sheets summarizing the new International HHT Guidelines.

Each fact sheet contains:

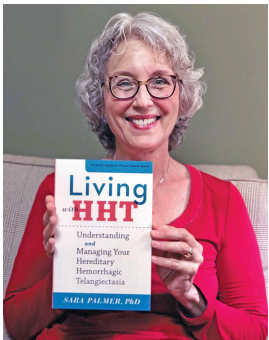
- Signs and Symptoms
- Important Facts to Remember
- How HHT is Diagnosed
- Treatment, and
- Discussion Points for You and Your Physician



The **Fact Sheets** have corresponding "My HHT Care Checklists" which is 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.

If you did not receive your complimentary copy from our Year End mailing, please connect with us and complete our Join Us form www.curehht.org/joinus to request your copy. Additional copies are available to order for our domestic and international community in our Cure HHT Store.

For those grappling with the emotional side of having an HHT diagnosis, we highly recommend **Dr. Sara Palmer's** book, *Living with HHT*. Autographed copies of Dr. Palmer's book are also available in our store and come with a **FREE** bookmark.



The Cure HHT Store

As Awareness month approaches, check out the Cure HHT Store for all your Awareness gear including our new 30th Anniversary T-shirt, face masks, wristbands and so much more. Don't miss this opportunity to spread a little bit of HHT Awareness right in your own hometown.

www.curehht.org/curehhtstore

Living with HHT - By Dr. Sara Palmer available in the Cure HHT Store and through Amazon Smile. Choose Cure HHT as your charity of choice.



Advocacy

Our Past, Present and Future

2020 was a year defined by a pandemic. As an organization, we stayed steady and strong through the most uncertain of times and positioned ourselves out of the shadow of COVID-19 in 2021 with the most vigorous advocacy approach in the history of our organization. If this past year has taught us anything, it is that our resilience, hard-work, and strength knows no bound. We will NOT be derailed in our mission.

With your help, Cure HHT has planted deep roots over the past 30 years by investing in basic science to transition into viable research, identifying and collaborating with the best scientists and researchers across the globe, and aligning the voice of the HHT patient with research to address the most critical needs and goals. 2021 will reveal Cure HHT's unwavering diligence in bringing HHT out of the darkness of the rare disease community and creating opportunities for advancement of the most viable therapies to date.

OUR ADVOCACY STARTS HERE

- ◆ Educate healthcare providers worldwide about HHT
- ◆ Form partnerships that fuel the mission of Cure HHT
- ◆ Engage and empower our HHT Community
- ◆ Develop and gain support from Government agencies.



AAFHHT
Asociación Argentina
de Familias con HHT



NATIONAL HEMOPHILIA FOUNDATION
for all bleeding disorders



PHA Pulmonary Hypertension Association
Empowered by hope



sanguina

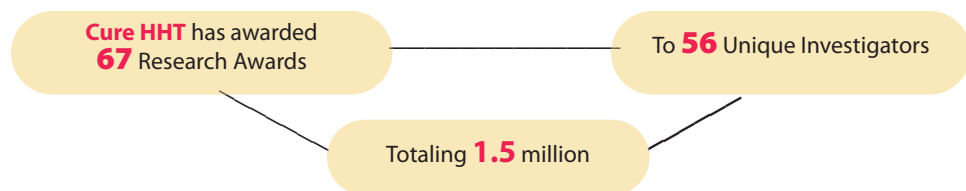
RESEARCH

Cure HHT's mission of finding a cure for HHT while saving the lives and improving the well-being of individuals and families affected by HHT continues to drive our biggest advances ever.

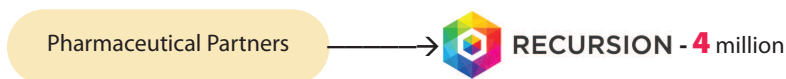
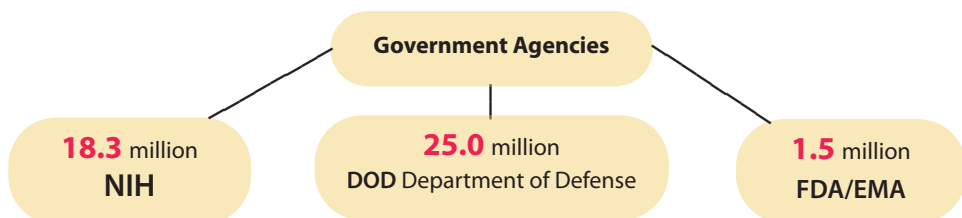
Through the years, Cure HHT's seed grants have leveraged funds to secure our most innovative research and education to date. This combined funding has created basic science and clinical research, state-of-the-art educational opportunities, and expanded access to care through our Centers of Excellence and now, our newest HHT-HTC Treatment Centers. We have identified the success of these initiatives through our steadfast and persistent advocacy. From our first seed grant in 2003 to fund a research project to our consistent and ongoing efforts on Capitol Hill, these initiatives have helped us gain the experience to recognize that funding for medical research must be robust, sustained, and predictable.



BY THE NUMBERS



Your donations of **1.5 million** created seed money that secured **48.8 million** of piggy-back funding



Totaling 50.3 million in funding our mission to find, treat and cure HHT

FUTURE AT A GLANCE

As we review our alignment over the past three decades of research partnerships and the continued advancement of effective therapies for our patients, our commitment to our global community remains fierce. We step boldly into the future as we:

- ◆ **Advance therapies and offer clinical trials** through our work with our global community of physicians and scientists
- ◆ **Expand genetic testing** through the Rare Genomes Project
- ◆ **Continue funding research** for HHT through the renewal of the “vascular malformations” designation in the PRMRP through the Department of Defense
- ◆ **Secure government funding** for our Centers of Excellence to expand operations, efficiency, diagnosis, and treatment
- ◆ **Provide educational opportunities** for health care providers through medical professional organizations

Advancing these initiatives will require significant additional investment, new strategies, and leveraging our resources to secure government funding. We are up to the challenge but need your patience, dedication, and unwavering support. *All of our efforts stem from the needs of our HHT Community—the heart and soul of Cure HHT.*

Your tax-deductible contribution, in any amount, helps us plan, build and fund so many of our initiatives. To help us expand our reach, join our host of monthly donors by making a monthly gift - \$10, \$25, \$50, \$100 or more. Simply check the box “Make my gift monthly” on the enclosed donation envelope or donate online. www.curehht.org/ways-to-donate *Thank You!*

Cure HHT Research Network Update

THANK YOU to everyone who participated in the Research Network surveys! As we read through responses from our patients, clinicians, and scientists we are learning so much about what is important to our community and how the Cure HHT Research Network can help to fill the gaps in HHT health. By this time next year, we will have a fully developed HHT Research Roadmap that outlines our priorities for funding HHT research, based on how it will contribute to our goal of improving outcomes for the HHT patient. We are SO enthusiastic about this project and are confident that incorporating the patient voice to guide research will change the landscape of HHT health.



**CURE HHT
RESEARCH NETWORK**

Keep an eye out for updates on this initiative in the Cure HHT electronic Newsletter. If you have any questions or ideas, please reach out! We love to hear from our community (it is your voice that drives us), just send us an email at researchnetwork@curehht.org.



A Deep Dive into Your Genetics!

Have you always wanted to explore a genetic HHT diagnosis or had genetic testing that resulted in an “inconclusive” outcome, yet

you have symptoms of HHT? Now's your opportunity to explore your genetics in depth through an exciting new partnership with Cure HHT and The Rare Genomes Project, created through the Broad Institute, a Harvard/MIT Group, that will provide patients just that opportunity.

The Rare Genomes Project is a patient-driven research project that performs full genome sequencing at no cost to families with suspected rare disease who do not have a genetic diagnosis. The goal of their project is two-fold: (1) provide access to testing for families that are genetically undiagnosed, and (2) discover previously un-identified gene mutations that may lead to disease.

Families who could benefit from participation are those who fit either of the following criteria:

- Have not had genetic testing done and do not have access to genetic testing.
- Have had genetic testing done and the results were inconclusive.

Note: a clinical diagnosis of HHT is not required to participate nor does it exclude someone from participating.

Participation is completely remote and FREE!

Just visit <https://raregenomes.org/how-it-works> or call 617-714-7395 to learn more!

Message from our Chief Medical Officer

Defining HHT: Is HHT a Bleeding Disorder?

This is a question that has come up frequently in the last few months. People have been asking if HHT is a bleeding disorder. There has been some question and often debate about the correct answer.

The short answer is yes, HHT is a bleeding disorder but it can be confusing. For those with HHT, and their families, it would seem fairly straightforward. People with HHT bleed. The cause for bleeding in HHT is what seems to be leading to the debates about this issue. There are many reasons people bleed. Injuries, stomach ulcers, medications and many more problems can lead to bleeding. People with HHT bleed because of abnormal blood vessels that develop in the skin, nose, GI tract and elsewhere. Some people state that HHT is a blood vessel disorder and others claim it to be a bleeding disorder. Both are correct. HHT is a bleeding disorder caused by a blood vessel disorder.



Scott Olitsky, M.D., M.B.A.
Chief Medical Officer

This is not just an issue of semantics. It is important. First, it is accurate and we should strive to accurately describe HHT and how it affects people who have it. **Second, HHT is the second most common inherited bleeding disorder yet it receives far less funding and research support than other inherited bleeding disorders that are far less common such as hemophilia.** One goal of increasing awareness is to rectify this imbalance and we can help do this by properly classifying HHT as a bleeding disorder and advocate for the support it deserves.

Should people with HHT be concerned about receiving a COVID-19 vaccine?

The concern about the vaccine is that the deeper injection could lead to bleeding at the injection site in people who have problems of blood coagulation, meaning people who have disorders where their blood does not clot normally, and they could potentially bleed significantly where the injection is given.

Because people with HHT do not have problems with coagulation of their blood, the injection is safe for the typical person with HHT. If you have HHT and are on a medication to alter your coagulation (such as coumadin for atrial fibrillation), you should check with your doctor. The warning should have been given for people with coagulation problems. Most likely the term bleeding was used because this is the more common lay term and is often considered to be synonymous with the word "bleeding" even though they are indeed different. The fact that the word bleeding was used has led to the confusion regarding HHT is indicative of the work that needs to be done to raise awareness of HHT, even among medical professionals.

Research Update

If you participated in the "Randomized Controlled Trial of Bevacizumab for HHT-Related Epistaxis" (aka "Bevacizumab (Avastin) clinical trial") at Stanford (2014-2019) and are interested in learning which intervention you received (drug or placebo), please contact Erik Chan at erikchan@stanford.edu.

Kids Making an Impact

Nothing warms our hearts more than witnessing the power of the kids in our HHT Community. Read how these four dedicated, kind and passionate miniature superstars are making a difference!

Gabrielle (Gabi) and Gabriella - Bronx, NY



In March 2020, after their school abruptly closed due to one of the first identified cases of COVID-19 in the US, these two BFF's Gabi and Gabriella quickly figured out a way to make use of all their free time. The girls heard about "mask wearing," to help combat COVID-19 and cleverly

designed and sold unique beaded mask holders, donating all their proceeds to Cure HHT in honor of Gabi's sister who has HHT. Kudos to these empowering young ladies with the biggest of hearts!



Cayleigh - Omaha, NE



To meet Cayleigh is to love her. Cayleigh dreams of the day she could own a candy store. Mom, Danielle, wanted to teach Cayleigh a lesson about candy store ownership and turn it into a way to give back. Cayleigh designed and made homemade Valentine's Day candy to sell—all to benefit Cure HHT, a disease she fights every day. Bravo Cayleigh! We hope your dream becomes a reality!



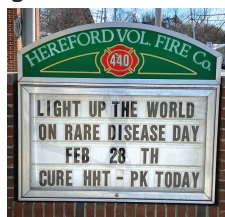
Oscean - Upperco, MD



This past February, Rare Disease Month, Oscean decided to shed light on rare disease in his own special way. Oscean had learned a lot about rare diseases, particularly HHT (his Mom is a Cure HHT staffer), and he wanted to put his knowledge to work in spreading awareness.

Following NORD's (The National Organization for Rare Disease) call to action to light up landmarks for Rare Disease Day, Oscean rallied his local politicians for support. His efforts resulted in the lighting of the Maryland State Government House, his school campus, the local fire department, and his church.

Oscean's inspiring project will be featured in next month's *Scouting Life* (formerly *Boy's Life*) magazine which has an audience of 2.1 million subscribers. Now that's how you spread awareness. We can't wait to see more "Good Turns" for HHT from this young Scout!



Strides for Strength is Back!

Our 2nd Annual Global Walk for a Cure – *Strides for Strength* -is back for another exciting year and we are doing things BIGGER and BETTER than ever before!

Grab your walking shoes, round up your friends and family, and leash your furry friend to start clocking those miles for our largest show of strength this year. Register to walk along or form a team, set your fundraising goal, and start walking your way to earning some cool incentives and prizes. Best of all, you'll be walking your way to further our mission to find, treat and cure HHT!

There are multiple ways for you to participate and support the HHT Community: become a sponsor, donate, fundraise, or walk! Mix and match these options however you'd like and motivate your friends to join too! Each registration includes a free "HHT Strong 2021" – *Strides for Strength* sticker. Donations and registration fees (excluding event T-shirts) are 100% tax deductible.



Indiana
Hemophilia
& Thrombosis
Center

Strides for Strength is proudly
sponsored by IHTC

Start walking towards a cure. Register today!

www.curehht.org/strides-for-strength

June Awareness Month and Cure HHT 30th Anniversary Celebration

Mark your calendar for a June that is packed full of awareness, celebration and more. We don't want you to miss a single moment.

June 1st – June HHT Awareness Month Kickoff

in front of our giant HHT Billboard. FB Live – Instagram Reels

June 5th – Strides for Strength Starts

Kick off event with last year's mile winner, Robert Schmidt.

June 20th – Strides for Strength Ends

All miles must be entered by June 21st.

June 23rd – HHT Awareness Day

Cure HHT's 30th Anniversary Celebration Virtual Event

Details and Ticket Registration and Purchases at www.curehht.org

June 30th – \$30.00 for 30 Years – Day of Giving

For all event details, visit www.curehht.org



Cure HHT
PO Box 329
Monkton, MD 21111



**Don't miss our *HHT International Guidelines*
online Education Sessions – Register Today!**

May 25 - Lung AVMs

June 8 - Liver VMs

June 12 - Nosebleeds

June 24 - Diagnosis

TBD – Nosebleeds (Featured in Spanish)

Visit www.curehht.org/online-education
to register for current sessions or to view past sessions.