



Edition:  
Fall-  
2022

# Cure HHT newsletter

News & Information for the HHT Community

## *Hope Is On The Horizon*

The HHT community has reason to be optimistic. Undeniably, much work remains: the road to finding a cure will be hard-fought. But it's a journey for which we are more prepared than ever before, as many factors are converging to allow us to believe that breakthroughs are possible in the not-so-distant future.

We feel this way for several reasons. First and foremost, because of you – our supportive and mighty community. Hope would not exist without your continued support. We recently concluded a tremendously successful June Awareness Month campaign, and a full recap can be found in this newsletter. Your generosity makes an impact in the lives of patients everywhere... and is the fuel behind our reasons for hope.

Made possible by your support, scientific advancements are accelerating. The PATH trial continues to recruit with promising results to date, and you can read more about our soon-to-launch Pazopanib trial – for which we have great excitement. These are milestones we could have only dreamt of a few years ago. In addition, born out of the Cure HHT Research Network (CHRN), our roadmap of research priorities is under development and will focus the brightest minds in HHT in a way that will lead to true impact.

Given all of this, our organization is also adapting to ensure we are able to turn “hope” into impact. Within this edition, read more about our new Therapeutic Development Arm – which has been created to give us the structure and expertise needed to set our sights higher and drive true transformation.

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## Message from Marianne

### **T**he Time Is Now: Adapting Our Organization For Impact

In this newsletter you will learn more about our new Therapeutic Development Arm – an investment we’re making to help accelerate and own the search for better treatments... and one day, a cure. More specifics are in the following pages, but I wanted to share why this is the right move at the right time.

Over the last decade, our goals have been to increase awareness, expand access to care, develop resources to help patient and physicians, and provide grants for promising research. We’ve made tremendous progress towards those goals.

For example, there were just 10 Centers of Excellence (CoEs) a decade ago. Today, there are more than 30. Then, the Department of Defense (DoD) was not investing in HHT... today, it has funded more than \$26 million in HHT research. Externally, the biotech space has exploded and the Food and Drug Administration (FDA) is giving companies exclusives for rare disease research. As a result, breakthroughs are happening faster than ever.

We have been building toward this moment since our founding. Enough is enough! The time is now. Breakthroughs are happening for other rare diseases, like sickle cell and hemophilia. Why not us? This will help us create a tomorrow where therapies manage chronic aspects of HHT, not surgeries. Where we can have a quality of life our parents couldn’t. A tomorrow where our children are cured.

For me, I have grandchildren I want to meet. I want a future where this disease will not take me from that. All patients have a similar hope. The steps we are undertaking now make that future possible.

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](mailto:marianne.clancy@curehht.org)

### Cure HHT Board of Directors Updates

Excited to announce **John Dunn**, on the board since 2018, has now been named as President of the Board. Special thanks to past president **Sara Palmer** for nearly 8 years of leadership, who will continue on the board. Also, **Savanah Schott** will take over responsibilities as Secretary. Visit [www.curehht.org/who-we-are/leadership-board/](http://www.curehht.org/who-we-are/leadership-board/) to learn more about our leadership team.



John Dunn



Sara Palmer



Savanah Schott



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Stay current on HHT clinical trials, education programs and community events. Sign up to receive electronic announcements by registering at [curehht.org](https://curehht.org) or send us an email at [hhtinfo@curehht.org](mailto:hhtinfo@curehht.org)

### CURE HHT NEWSLETTER

**Marianne Clancy, RDH, MPA**

*Editor*

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# UF Health Named **31st HHT Center of Excellence**

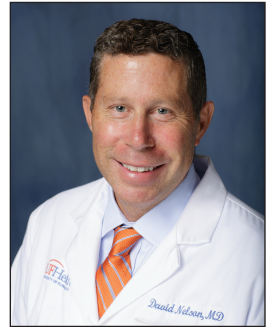


**Cure HHT is excited to announce UF Health has been named the first HHT Center of Excellence in Florida and the 31st in North America!**

Cure HHT recognizes centers equipped with the personnel, expertise, commitment and resources to provide comprehensive evaluation, treatment and education to individuals with HHT and their families. Recognition as an HHT CoE follows 18 months of training, mentor center communication and site visits from the team at Cure HHT.

*"It can be challenging for people with rare medical conditions to find the specialized care that can transform a life and ease the burden of chronic illness. We're proud to be able to offer that expertise at UF Health and thank the Cure HHT foundation for designating UF Health as a center of excellence in the treatment and research of this insidious disease."*

- **David Nelson, M.D.**, UF's senior vice president for health affairs and president of UF Health.



Under the leadership of CoE Director **Dr. Ali Ataya**, the specialties that support care through the UF Health HHT Center of Excellence include neurosurgery, interventional radiology, pulmonology, gastrointestinal/hepatology, neuroradiology, ENT, cardiology and genetics.

***Accepting new patients,  
call: 352-273-8990***



# So, What Goes into Establishing A New Center of Excellence?

By Scott Olitsky, *Cure HHT Chief Medical Officer*



**When a new HHT CoE opens, our entire community benefits.** It means more physicians are working to understand HHT and advance treatments, and it enlarges the pool of clinical research. It also boosts the number of young investigators and trainees who learn about HHT early in their careers.

With several new centers launched recently, I wanted to shed some light on the process.

It involves several rigorous steps from our staff at Cure HHT – much of which happens behind the scenes.

It usually begins with a group of physicians who have an interest in HHT, often because they have trained at an already-established center. As their experience increases, they often increase their interactions with our community by attending our webinars, meetings, etc. At some point, a goal of becoming a CoE is discussed and an application is completed. Cure HHT examines the application to ensure the level of expertise needed is present and assigns a mentor center. Several conference calls are hosted and a visit to the mentor center is made to see how a CoE works best. The mentor center compiles a post-visit report for us, outlining any potential concerns. If there are concerns, we work to address them and the application may be put on hold until rectified. Once addressed, our team visits the institution and interviews the prospective CoE staff. A report is then made, and any concerns that were found are again addressed. When everything is felt to be satisfactory, the process moves forward with a recommendation to the Cure HHT Board of Directors -- who provides the final approval.

While the above is a quick overview, it is by no means a short process. Countless hours go in to making CoEs happen. While patients understandably want to see new centers added, it is important that the centers are added only when they are ready. This may delay, or even eliminate, the opening of some proposed centers.

However, increasing access to specialized care remains a top priority for our organization, and **we are proud to have opened more than 20 new centers in just the last decade!**

# Owning Our Future: **Introducing Our New**

**Our mission has remained the same since our founding: to find a cure for HHT... to create a future without the unnecessary suffering, without the pain and loss.** While much work remains to make that future a reality, we're excited to introduce a monumental step we're taking to bring that future forward faster.

To help us more directly create transformative medical impact, we've created a new **Therapeutic Development Arm of our foundation.** This will allow us to be more proactive in accelerating knowledge, developing therapies, and moving faster to find a cure. With scientific advancements happening more quickly than ever before, this team will give us the in-house expertise to harness scientific opportunities and turn them into impact for HHT patients everywhere.

This team is responsible for managing our Centers of Excellence strategy, owning patient registries, establishing strategic partnerships with pharmaceutical companies, overseeing our portfolio of research, and fostering greater collaboration among our scientific community.

Several factors are converging to make this the opportune time for such a move. Internally, we have made significant progress in expanding the number of physicians and researchers focusing on HHT. Externally, scientific breakthroughs are materializing faster than before.

While we're placing enhanced focus on driving science forward, supporting our community and increasing awareness and education remains a critical part of our mission. We're pleased to announce that longtime staff member Nicole Schafer has been elevated to Chief Operating Officer and will now oversee our educational programs, community engagement, donor development, and more.

Our organization needs the expertise to harness the power of the community we've built and the science that's possible today to launch effective therapies faster.

**We cannot sit on the sidelines and hope researchers are investing in our disease. We must make it happen -- and ensure our patient voices are at the center of it all.**

# Therapeutic Development Arm

This transformation would not have been possible without the support of the **Chan Zuckerberg Initiative (CZI) "Rare as One" grant**. In 2019, we were one of only 30 rare disease organizations selected. The foundation has equipped us with the confidence to grow our patient-centered research infrastructure in a way that will create true advancement.

We are setting our sights higher than ever before because we believe real hope is within reach.

## Therapeutic Development Arm Structure:

- Senior Director of Research & Strategic Medical Partnerships  
(Marianne Clancy)
  - Director, National CoE Coordinating Center  
(Melissa Dickey, RN, MSN, FNP)
  - Director, Cure HHT Research Network Programs and Grant Management  
(Cassi Friday, MS, PhD)
    - Global CoE Strategy Director  
(Scott Olitsky, MD)
    - Project Manager, Pazopanib Clinical Trial  
(Gary Saum)
  - Patient Education and Registry Administrator  
(Courtney Kasturiarachi, MPH)
- National CoE Coordinating Center and CME Administrator  
(Nolie Krock, MPH)
  - Brain Vascular Malformation Consortium (BVMC)  
Clinical Research Coordinator (Leslie Perry, MSN, RN)



## STAND WITH US AS WE TRANSFORM OUR ORGANIZATION TO SERVE YOU!

Dear Friends - We will all look back on this organizational transformation with pride as we take a giant leap forward to harness science for our futures, and for our children's futures. Even with all of our past success, never before has Cure HHT been on the cusp of achieving so much.

While we remain a small, effective and efficiently staffed non-profit, our team is now poised to bring you major breakthroughs. Your tax-deductible donations, retirement distributions and legacy gifts will continue to help make the difference! Please use the enclosed envelope or visit [www.curehht.org/ways-to-donate/](http://www.curehht.org/ways-to-donate/) Thank you for giving as generously as you are able! - **Marianne Clancy, Cure HHT Executive Director**

# Our Community Is Incredible:

## Recapping Another Successful Awareness Month

Hope exists for HHT patients around the world thanks to our incredibly generous and committed community.



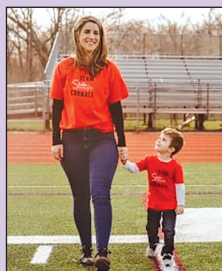
Through our Awareness Campaign and 3rd annual “Strides For Strength – A Virtual Walk for a Cure” event, we are thrilled to share we raised over \$178,000 to support our work to find, treat, and – one day – cure HHT.



Those that donated, walked, shared their stories on social media, and more... you’ve made a true difference. We are incredibly thankful for you! Your donations will be put to work in a multitude of ways – from supporting our international scientific conference to expand knowledge and research, sponsoring young investigator travel grants to inspire the next generation of HHT physicians, funding research and studies, and so much more.

## Strides For Strength by the Numbers

- \$75.4K Raised
- Top Fundraising Team:  
**Team Jeyren Marin** - \$7,623
- Top Fundraising Individual:  
**Juliana Cornale** - \$5,089
- 186 participants
- 27 States Represented
- 34 Pets participated
- 5 Countries participated



Team Jeyren Marin

Juliana Cornale



## Thank You to our Generous Strides For Strength Sponsors



**JB BLEVINS & FAMILY**

**The Competiello Family**

**Pat & Maijo McCune**

**The Village Carpenter**

**JOHN CAMPBELL**



# Increasing Knowledge Across Our Community

When it comes to HHT, knowledge is power. With up to 90% of people with HHT remaining undiagnosed today, heightening awareness among all aspects of our community – from patients to physicians to our staff – is a critical component of our work.

## Driving Clinical Advancements

Later this month, we will be bringing together leading clinicians and scientists from around the world at our **14th HHT International Scientific Conference** all in the name of expanding collaborations that accelerate progress in HHT research and treatment. Look for a full recap in our winter newsletter. Our generous community supports our efforts to expand HHT knowledge by donating to help young investigators attend, ushering in the next wave of HHT researchers and clinicians.



## Chan Zuckerberg Initiative Annual Meeting

Earlier this summer, members of our team at Cure HHT spent several days learning from leading experts in science and research, and collaborating with inspiring leaders from other rare disease organizations at the Chan Zuckerberg Initiative Rare As One Network Annual Meeting.



Many insights were gained, and we are leveraging this grant and resource to better unlock the power of patient-driven research for our community!



## 1st HHT Academy Virtual Conference

### Was a Smashing Success!

To help expand knowledge among both patients and physicians, we recently concluded our first ever **HHT Academy Virtual Patient and Physician conference**. The 9-day learning extravaganza featured courses led by some of the world's leading HHT experts and geared specifically for patients, international attendees and physicians. We hope those who attended found it valuable, and we are excited to share that the academy saw more than 120 new community members join us!

**Thank you to our generous sponsors:**

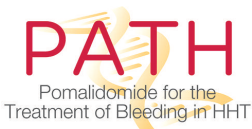
- **Nampons**
- **The Eckhauser Family**
- **The Drysdale Family**
- **Scott & Andrea Olitsky**
- **Vaderis Therapeutics**

# Pushing for Progress

**A critical part of our mission is serving as a driving force to advance better treatment and knowledge of HHT, to help us one day find a cure.**

## Recruiting For PATH Study

Recruitment for our PATH-HHT study is well underway at 10 HHT CoEs across the US with a few more centers being added, but we are still in NEED of additional patients to meet our study benchmarks.



With chronic nosebleeds impacting a large percentage of the HHT population, this study is vitally important in helping to develop better and more effective treatment

options to manage nosebleeds. To learn more, visit [www.curehht.org/clinical-trials/](http://www.curehht.org/clinical-trials/)

## Coming Soon: Pazopanib Clinical Trial

Soon, we will begin recruiting for a tremendously promising clinical trial of a drug aimed at treating bleeding, called Pazopanib. Our goal is to obtain an FDA registration for approval for this to be our first approved HHT therapy drug. Scientific breakthroughs are accelerating, and this trial marks an exciting milestone in advancing treatments.

## Brain AVM Study – *Participants Still Needed*

Centers are still recruiting patients to participate in an NIH funded study, entitled “Cerebral Hemorrhage Risk in 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.



## FREE Genetic Testing For Rare Disease

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/](http://www.curehht.org/rare-genomes-project/)



# Our Voices Are Powerful

**June Awareness Month** may be over, but our mission to heighten awareness is a year-long one. To make true progress, it takes all of us.

## Spreading Awareness 1 Mile at a Time

"What is HHT?" It's a question **Marijo McCune** gets asked often and it's exactly what she hoped to accomplish when she made the decision to create a license plate that reads, "**CURE HHT**."



"Though I may never know how many people have asked Google [What is HHT], I'm sure there's been many. They say curiosity is the engine of achievement. With this achievement, we create awareness. It's a simple thing we all can do," says Marijo.

Never underestimate the power one conversation, one social media post or in Marijo's case, one license plate can have.

Visit our blog to read Marijo's full story and several other new patient stories. Submitting your story is a powerful way to help us continue to spread

awareness! [www.curehht.org/blog/](http://www.curehht.org/blog/)

## Big Wins for HHT Awareness!

It's estimated that up to 90% of HHT patients today remain undiagnosed, and our community continues to play a powerful role in changing that! We want to give shout-outs to the **Hearts for Haley Foundation** and the **Jordyn and Young families** for their great awareness-raising efforts!

The **Morris family** recently organized an event at a **Phillies baseball game**, where Haley's story was displayed on the jumbotron and her brother threw out the first pitch!

As for the **Young family**, they entered an amazing **HHT Float** in their local **Fourth of July Parade**!



***Together, we can make a difference!***



**Cure HHT**  
PO Box 329  
Monkton, MD 21111



## **Guidelines and Checklists Coming Soon In Spanish**

Our HHT guidelines and checklists are comprehensive resources to help you navigate the complex care HHT requires – and they'll soon be available in Spanish. Visit our website soon to get your copies.

If you no longer wish to receive this newsletter please contact [hhtinfo@curehht.org](mailto:hhtinfo@curehht.org) or 410-357-9932.