

Edition:
Spring
2020

Cure HHT newsletter

News & Information for the HHT Community

HHT Physicians and Patients Collaborate to Define the Best Care Around the World!



Hosting the Christopher McMahon Memorial International HHT Clinical Guidelines Conference in Toronto, Canada, was a “pinch-me” moment for us here at Cure HHT. Our steadfast vision, and determination to join the voices and experience of HHT specialists and patients from around the globe, with a singular goal—to update critical HHT guidelines, finally came to pass in Toronto, Canada, and was the absolute success we only dreamed. The dedication of our Cure HHT Team, along with the generous support of the Christopher McMahon family, brought together over sixty-five HHT specialists and patients to the “Queen City” to create the most up-to-date guidelines for HHT screening, diagnosis and treatment. The critical material gathered from this in-depth

study of current treatments, protocols and best practices will have the ability to impact every person undiagnosed or diagnosed with HHT. This means you and your family - members of the global HHT community! Stay tuned to your mailbox, inbox and the Cure HHT website for the much-anticipated publication of these essential guidelines coming in a few months.

All of our success is YOUR success and your support continues to help us know NO limits!

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Message from Marianne

I planned a different type of message as we were putting together this first newsletter of 2020, but then our world quickly changed in the face of a global health epidemic ("pandemic"). As everyone continues to adapt to a new normal for a while, we have greatly missed seeing you at our Spring events and conferences. What has not changed, however, is our commitment in providing you and your families with the most important information affecting your health and the COVID-19 crisis. We are working daily to fulfill our organizational mission.

As the hub for all HHT information, we reached out to our trusted team of physicians and experts, and sent important medical information to you through our E-News, special emails and social media posts.

Answering your important questions about specific issues related to HHT and the virus has been a priority. As your Executive Director, I even hosted my first Facebook Live to provide the latest Cure HHT research and medical updates. Continue to check back through all our communication platforms as we provide weekly updates. You can rest assured we are committed and passionate about providing accurate information. Living with a chronic illness during this pandemic presents us with additional challenges. The importance of staying connected is critical.

Looking to the future and as we come through this crisis together, I reflect on when our organization was founded in 1991. We have grown as a strong and resourceful community, making transformational change with the way HHT is diagnosed, treated and managed—you can count on us to help produce outcomes for greater quality of life. With that comes the announcement of the new Chan Zuckerberg Initiative "Rare as One" grant, which is focused on development of a Research Network led by the patient voice for rare disease. I am thrilled to share all the exciting details of this exceptional opportunity (see pages 6-7). This is our future and I invite you to join us in the HOPE that together, we will continue to invest in research with the urgency required to transform this disease for all of us and our children.

Be safe and well everyone and #StayHome!

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

Thank You and Farewell !



We would like to take this opportunity to share our heartfelt appreciation to the former Cure HHT Board President, Chandra McMahon, who steered us into this pivotal stage of our organization. We are eternally grateful for Chandra's steadfast leadership throughout the years and the invaluable contributions she and her family have made to our organization, particularly their selfless gift of funding the important Christopher McMahon Memorial International Guidelines Conference this past November. *We wish Chandra all our best!*



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<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

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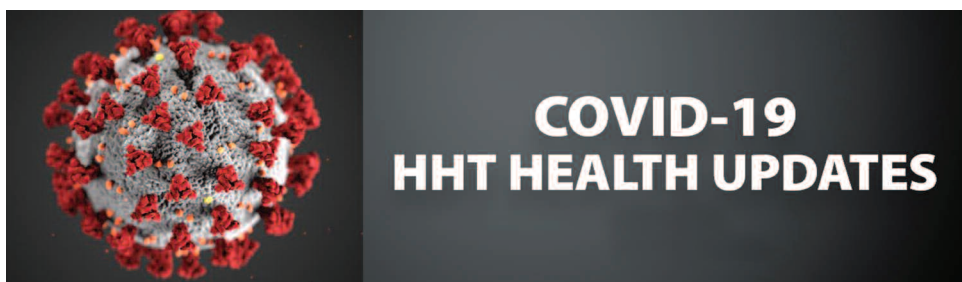
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Special Announcement from Cure HHT Executive Director, Board of Directors and Staff



Two years ago, we coined the phrase #InThisTogether as part of our annual year-end appeal. Now more than ever, this simple phrase has been on our minds and is driving all that we do during these unprecedented times surrounding the outbreak of the novel Coronavirus (COVID-19). The impact COVID-19 has had on our communities, HHT patients, physicians, and Cure HHT staff has been something we never thought we'd encounter in our lifetime. With that being said, we wanted to connect with you directly and to put your mind at ease knowing that we are truly #InThisTogether with our entire HHT community as we continue to navigate uncharted territory, not only as an organization, but as a community.

First and foremost, the well-being and safety of our HHT patients, healthcare providers and Cure HHT staff is our top priority and will always be at the heart of every decision we make. In that regard, we have taken these important measures: We are following and reporting to our HHT community the continuing updates and recommendations of the Centers of Disease Control and Prevention ("CDC") and the World Health Organization ("WHO") in order to provide you with reliable information surrounding the best practices in avoiding a COVID-19 diagnosis. The CDC has issued the 5 "To Do's" (washing your hands, not touching your face, social distancing, staying home if feeling ill, and seeking medical attention if you become symptomatic). It is important that each of you follow your local state government health department's recommendations concerning personal hygiene, social distancing, and seeking medical treatment above all else. Your local HHT Centers of Excellence (COEs) will also be following their government's recommendations, which will trickle down to you and your treatment.

We are actively engaging with our physicians at our North American COEs, keeping you informed of the necessary precautions and treatment protocols in place, if you are exposed or contract the virus. We fully support our COEs in their well-thought out decisions regarding routine scheduling of procedures and treatments during this tumultuous period. Our COEs will continue to monitor and evaluate HHT patients either virtually or in-person for necessary treatments where suspension of treatment could potentially cause life-threatening situations. Our COEs are adhering to their institutions' policies revolving around COVID-19. Please know that each of these decisions were made with their patients' best interest in mind. While some COEs have cancelled non-emergent appointments over the coming weeks, they stand ready to reschedule those appointments the moment they receive the go-ahead from their hospital administrations and know that their patients are at a significantly reduced or non-existent risk for COVID-19. Please check in with your local COEs for individual direction regarding center visits.

Taking into consideration the recommendations of the CDC and many local and state-wide restrictions on public events, and to adhere to all social distancing strategies in place, we have temporarily suspended all of our Cure HHT sponsored events across the nation, which includes our Walks, Patient and Physician Conferences and Walk and Talk Series. Please know that we are as disappointed as you that we had to take these measures and are unable to connect this spring in person. We know that this is the biggest way, as an organization, we can help reduce the spread of this virus.

In the meantime, we are a fully functioning staff and are moving forward with all of our exciting 2020 plans including more clinical trials, dedicated research focused on new treatments, overhaul and revamping of our Resource Library, the opening of new HHT Centers of Excellence to add to our existing twenty-six COEs in North America, educating medical professionals about HHT, hosting informative Webinars on topics of interest in our HHT Community, creating patient advocacy groups in your local communities, and connecting daily on all six of our social media platforms (Facebook, Instagram, Twitter, Vimeo, YouTube, LinkedIn).

Now more than ever, we encourage you to connect with us through each of our communications--email, social media, webinars, and even good old snail mail! Fill out our registration form at curehht.org/get-involved/stay-in-touch/ so we can better serve you. We don't want you to miss a single announcement, resource, recommendation or connection. We are here for you every step of the way!

We are busier than ever and are not slowing down. We won't stop, even during these unsettling times as we owe it to our patients—the reason behind everything we do. Our goal over the coming weeks, while this situation continues to evolve, is to remain consistent, transparent and a place of solace and hope for our HHT Community. For all information COVID-19 related, visit our website at curehht.org/covid19 Updates are added weekly, every Thursday.

BOARD ANNOUNCEMENTS



Dr. Sara Palmer



Ben Holt, Esq.



Cheryl Wilson



Karen Weissel, Ph. D.

Many of you already know **Dr. Sara Palmer** as a patient and author of *Living with HHT*. Now, Sara will be taking on a new role as our Board President. Sara is joined by **Ben Holt, Esquire** as our new Board Vice-President, both appointments effective November 1, 2019. Sara and Ben will make an incredible team as they lead Cure HHT into the new decade. We also welcome new Board members, **Cheryl Wilson** and **Karen Silber Weissel, Ph. D.**, who bring their HHT knowledge, talent and professional insight to fostering the Cure HHT mission. To read more about our dedicated group of Board Members, visit: <https://curehht.org/who-we-are/leadership-board/>

Rare^{As}ONE

Cure HHT to Launch New, Patient-Led Research Network in Collaboration with the Chan Zuckerberg Initiative

We are 1 in 5,000 and Rare as One! Our excitement knows no bounds – Cure HHT has been selected for a highly competitive, two-year grant from the **Chan Zuckerberg Initiative's Rare As One Project!**

The goal is to develop and launch a collaborative, patient-led research network in partnership with clinicians and researchers from around the world! This unique opportunity will provide Cure HHT with funding, tools, expansion support and training to create a larger, more integrated HHT community. *But most importantly, this program will accelerate Cure HHT's mission to find new treatments and a cure for HHT!*

"No one is more committed to finding cures for rare diseases than the patients and families of those affected by these disorders. We are proud to support patient-led organizations as they pursue diagnoses, information, and treatment options in partnership with researchers and clinicians."

*–Priscilla Chan,
Co-Founder & Co-CEO of CZI*

Founded by Dr. Priscilla Chan and Mark Zuckerberg in 2015, the Chan Zuckerberg Initiative (CZI) is a new kind of philanthropy that's leveraging technology to help solve some of the world's toughest challenges — from eradicating disease, to improving education, to reforming the criminal justice system. CZI's Rare As One Project aims to support and elevate existing patient-focused communities like Cure HHT in order to drive progress against rare diseases. There are 7,000 diseases considered as rare, and Cure HHT is **one of only 30 organizations** selected to participate!

HHT Treatment Centers & Global Opportunities

-  **North American CoEs**
-  **International Centers**
-  **Expansion - next 5 years**

***WE WILL NEED YOUR VOICE TO MAKE THIS
RESEARCH NETWORK A SUCCESS!***

[Learn More:](#)

CureHHTResearchNetwork.org

ChanZuckerberg.com/science/programs-resources/rareasonel/



WHAT DOES THIS MEAN FOR OUR HHT COMMUNITY?

Marianne S. Clancy, Executive Director of Cure HHT explains, “Since this is a patient-driven network, its decisions will provide the greatest impact for patients.” Having a research network will allow Cure HHT to better leverage donations and existing grants; enlist clinical trials; foster new partnerships with biotech, pharmaceutical and device companies; and propel HHT research toward FDA-approved treatments.

This new **Cure HHT Research Network** will be led by **Marianne Clancy**; **Chris Hughes, PhD**, University of California, Irvine; and **Vivek Iyer, MD**, CoE Director Mayo Clinic, who will collaborate with HHT patients, scientists and physicians to ensure that the patient voice is heard, help identify gaps in current research, and develop plans to address the most critical needs.



CURE HHT RESEARCH NETWORK LEADERSHIP TEAM



Marianne Clancy,
Principal Investigator

Nicole Schaefer (left)
Nolie Krock (right)

“Kudos to CureHHT for successfully competing for the prestigious inaugural CZI award. The CZI award is a very important milestone for our HHT community and will help raise awareness and advance clinical and research initiatives and collaborations amongst basic scientists, clinicians and patients.”

— Vivek Iyer, MD



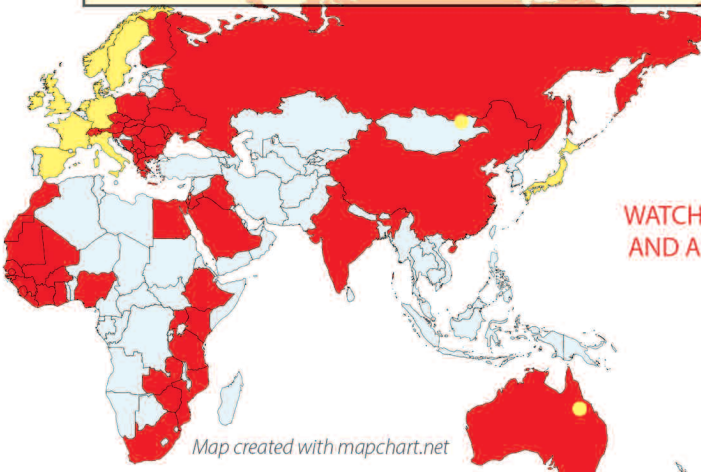
Vivek Iyer, MD
Lead Clinician

“This award is incredibly exciting news for us all. Thanks to the inspiring leadership of Marianne Clancy and the tireless work of her team, we now have the seed money we need to build a bigger and better advocacy organization. In particular, we will redouble our efforts to recruit the best and brightest scientists to work on HHT, as it is basic research that will drive us toward better treatments and a cure.”

— Chris Hughes, PhD



Chris Hughes, PhD
Lead Researcher



WATCH FOR MORE NEWS
AND ANNOUNCEMENTS
IN 2020!

New Treatment Study Shows Hope for Nosebleed Sufferers

We love to share stories of promising new HHT treatments on the horizon, especially nosebleed treatments since 90% of the HHT patients battle nosebleeds. This important study, conducted by physicians at the *Centre National de Reference pour la Maladie de Rendu-Osler*, reports on a promising topical drug alternative for nosebleeds, Tacrolimus. Here's a few words from **Sophie Dupuis-Girod, M.D.**, one of the lead researchers, on the exciting discoveries made during her clinical trials of Tacrolimus, a topical nasal ointment for nosebleeds.



Improving treatment of nosebleeds in HHT is one of the challenges we sought to address. We decided to focus our clinical research here at the French HHT Reference Center on treatments already approved or being developed in the field of Oncology. Based on our collaboration with Sabine Bailly's group, working on the repositioning approach developed by screening the libraries approved drugs, we embarked on evaluating the efficacy of the drug, Tacrolimus, which is a topical nasal ointment, on nosebleeds. We carried out a double-blind setting a prospective, phase II multi-center, randomized study and enrolled fifty patients. The results of this study were encouraging showing a good tolerance associated with a significant improvement in epistaxis duration during the treatment.

We were fortunate to have these initial phases financed by a grant provided by our institution (Hospices Civils de Lyon) and by the patients' association (AMRO-France). We decided to pursue additional funding to do a "phase 3 study", allowing further testing of the drug on additional patients. We are excited for the next phase of our research in bringing simple but effective treatment options to patients worldwide, particularly in the area of nosebleeds.

To review the full article about exciting new therapies under investigation, click here:

<https://ojrd.biomedcentral.com/articles/10.1186/s13023-019-1281-4>

Current Clinical Trials

Time and again our HHT Community has shared their enthusiasm to participate in Research. As the only patient advocacy organization focused on finding a Cure for HHT, we listen to the desires of our patients, making research our TOP priority. This year—2020—our vision for Research is unprecedented and we are excited to be a part of some of the most promising clinical studies out there. While these studies are currently on hold during the pandemic, now is the time to learn more about how YOU can become part of this important next step in finding a Cure!

DOXYCYCLINE - An inexpensive generic medication used by physicians for decades to treat bacterial infections has displayed potent properties in blocking growth of new blood vessels inside and outside the body. Two HHT Centers of Excellence are currently conducting clinical trials to determine whether this drug can be used to control nosebleeds for patients with HHT.

PAZOPANIB - Investigational new drug for a promising therapy for chronic bleeding and anemia. This Cure HHT sponsored clinical trial will begin recruiting at several HHT Centers of Excellence in North America later this year.

POMALIDOMIDE – The PATH study is exploring the use of an oral medication called pomalidomide for the treatment of nosebleeds in HHT at eleven research centers across the United States. Adults suffering from HHT with moderate to severe nosebleeds (epistaxis) who require iron infusions or blood transfusions are eligible. During the 6-month study, patients might receive either pomalidomide or a matching placebo (sugar pill) in addition to their usual care.

PATH is funded by a grant from the National Heart, Lung and Blood Institute, and 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. Learn more at: <https://path-hht.org>

TIMOLOL - A study for the use of Timolol, a new nasal gel medication to treat nosebleeds, is underway at Washington University in St. Louis, MO.

To learn more about each of these Clinical Trials, log onto our website for all the details:

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

Help Expand Care Across North America!



For our year-end appeal, we challenged you to **#AdvanceTheCure** by raising \$150,000 to be matched by a generous group of donors. Close to 300 of you -- patients, friends, families, and medical and scientific professionals stepped forward with gifts large and small, surpassing our goal and

raising more than \$300,000 for Cure HHT. Our HHT community proves time and again that we can meet -- and even exceed -- challenges when we act as one.

Our Cure HHT mission challenges us to bring everyone quality and knowledgeable care through our North American HHT Centers of Excellence (COEs). With tremendous effort we have established 26 COEs across the U.S. and Canada. Still, there are underserved areas and some patients travel miles to get the care they deserve. Our goal is ambitious: **COEs in ALL 50 states and throughout Canada. But just think....we are more than halfway there!**

COEs don't pop up out of nowhere -- a hospital or university that wants to create a COE must prove its dedication, expertise and commitment to the HHT community. Cure HHT's guidelines are strict, and institutions are thoroughly vetted by our Chief Medical Officer, physicians at other well-established COEs, and our Cure HHT staff. Once established, the COEs are continuously monitored for quality by Cure HHT.

Help Cure HHT speed up testing, increase diagnosis, and reach everyone who needs care! We are thrilled that we currently have applications pending and close to completion in **Virginia, Arizona and Florida, with several more in the pipeline.**

None of this would be possible without YOU and your generous donations!

Thank you for using the enclosed envelope to make a gift today to help us continue our vital work. You can also donate online at curehht.org

Here are some other great, easy ways to give:

FACEBOOK

100% of all donations made through **Facebook** by **direct donation, a donation button, or private fundraiser**, go directly to Cure HHT. Facebook does not take a dime! These fundraisers not only help raise awareness for HHT, but also contribute to Cure HHT initiatives. We are extremely grateful and encourage you to keep up the amazing work!



amazon smile

Amazon Smile may be one of the easiest ways to support Cure HHT. Amazon Smile donates a portion of proceeds from your regular purchases, directly to the charity of your choice. All you have to do is log in through Amazon Smile prior to making your purchases and **choose Cure HHT as your charity** - easy peasy.



Tracy Kelly

We are thrilled to introduce our newest member of the Cure HHT Team, **Tracy Kelly, Fundraising Event Coordinator**. Tracy, who has a BS in Mass Communications from Towson State University, has a long history with event planning and community outreach through her various positions including her

most recent engagement with the March of Dimes. Tracy's enthusiastic personality and stellar communication skills make her the ideal candidate for working with our HHT Community in planning our annual walks, patient events, and assisting our community in bringing their fundraising ideas to life. Tracy will also be piloting a new program where she will be organizing community outreach groups in various cities across the country.

We asked Tracy what she likes most about Cure HHT and our patients and here's what she had to say, *"I'm so inspired to be working with such a passionate team here at Cure HHT. I can't wait to take part in the continuing effort to promote awareness and raise the funds that will, one day in the very near future, assist in finding a cure for HHT."*

Update on Cure HHT Events

Tracy, along with the rest of the Cure HHT Team, was so disappointed to postpone our Spring events and a chance to meet everyone in person. However, she is already working around the clock to reschedule our in-person events for the Fall. She is also creating some exceptionally fun VIRTUAL events over the coming months. Stay tuned to our social media platforms and the Cure HHT events page on our website for all the details as they become available!

If you are interested in getting involved and/or looking for simple ways to bring Awareness about HHT to your hometown, please reach out to Tracy at tracy.kelly@curehht.org

PayPal is an ideal way to donate! PayPal does not collect any fees for charitable gifts so Cure HHT receives **100%** of your donation! You can even set up recurring gifts! Learn more about the PayPal Giving Fund by visiting curehht.org/ways-to-give, or search "Cure HHT" or "HHT Foundation International, Inc." in the list of PayPal Giving Fund charities. **You are not required to have a PayPal account in order to donate.**



Cure HHT has a **Zazzle** store! Cure HHT receives a portion of the proceeds from any item purchased from the Cure HHT store.

Cure HHT receives **100%** of every **eBay for Charity** donation through **shopping, selling or direct donation** as part of the eBay for Charities program. You do not need a separate login from your regular eBay account to donate. Be sure to choose PayPal for the transactions that you wish to have donated to Cure HHT!





Cure HHT
PO Box 329
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June is HHT Awareness Month!

Stay tuned for all of the news!

**For more on how you can help
visit <https://curehht.org/awareness>**

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