CHORUS
Comprehensive HHT Outcomes Registry of the United States

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Comprehensive HHT Outcomes Registry of the United States (CHORUS)
Protocol Information

STUDY OVERVIEW

The purpose of this study is to develop a data collection registry with a large cohort of Hereditary Hemorrhagic Telangiectasia (HHT) patients that will be followed over time. This will allow us to better understand HHT, the symptoms and complications it causes ("outcomes"), and the impact the disease has on people's lives. We will collect long-term information about participants, allowing us to understand how the disease changes over time, and what factors can influence those changes. Ultimately, this should help improve treatments for the disease.

Another important goal of the registry is to have a way to contact people to participate in future treatment clinical trials and other research. As recruitment for clinical trials in rare diseases is challenging, the registry will be a centralized resource for recruitment for clinical trials.

We estimate that about 10,000 participants will take part in this study over a 10-year period. Participants will be asked to allow for the collection of information from their medical records to be entered into the registry. This includes demographic information, diagnosis information, HHT genetic testing results, family history, organ involvement, imaging results, lab results, treatment information, symptoms, complications, lifestyle and other relevant medical information.

Participants will also be asked if they would like to participate in "HHT Connect" to answer surveys with questions about their symptoms, self-management, quality of life, and knowledge of their disease. If interested in participating, they can sign up at HHT Connect - Cure HHT.

STUDY DETAILS

- All patients evaluated and diagnosed with HHT in the U.S. will be eligible to participate in the study.
- Participants will be required to establish care at an HHT Center of Excellence actively recruiting HHT patients.
- Additional travel and/or visits will not be required if you are currently being followed at an HHT Center of Excellence actively recruiting HHT patients, except for routine HHT care and management.
- **No additional travel is required. Only information will be gathered, which will take approximately** two hours of your time for the enrollment visit or call and **one hour of your time each year** following enrollment. There are currently no reimbursements being offered for your time.

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OBJECTIVES

Primary Objective: To develop a data collection registry to better understand the natural history of HHT and treatment outcomes. Longitudinal data will be collected both retrospectively and prospectively, with a focus on increasing the understanding of this rare disease, accelerating the development of new diagnostic and treatment options, and working collaboratively with clinicians who care for individuals with HHT to identify and address gaps in the system of care, especially those from underserved populations.

Secondary Objectives:

1. To recruit a cohort of HHT patients across North America. We will collect comprehensive baseline clinical, demographic, and lifestyle data. Additionally, we will collect annual outcomes data. Participant data will be entered in CHORUS. This will be a resource for future clinical translational studies and is necessary given the urgent need for natural history data in this disease, with characterization of clinical outcomes.
2. To measure the clinical outcomes of HHT prospectively and longitudinally and characterize their outcome determinants (demographic, environmental, lifestyle, comorbidities, medications, HHT-genotype, organ vascular malformations, etc.).
3. Correlate clinical data entered in CHORUS to patient-entered data entered in "HHT Connect" regarding symptoms (epistaxis, migraine, etc.), self-management, quality of life and patient knowledge of their disease.

INCLUSION CRITERIA

1. Diagnosed with HHT based on the Curacao diagnostic criteria or genetic testing.
2. Able to provide informed consent or informed consent via a parent or legally authorized representative due to their age or medical condition.

EXCLUSION CRITERIA

1. Unable to provide informed consent or informed consent via a parent or legally authorized representative.

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SCHEDULE OF EVENTS

Total duration	10 years or until close of study
Baseline Visit	In-person visit or virtual visit at a participating enrollment site
Follow-up	Annually via phone call or an in-person visit or virtual visit at a participating enrollment site
Other tests	No additional testing required, except for routine HHT care and management

CLINICALTRIALS.GOV

The study is registered with clinicaltrials.gov. More information on the study design and status can be found here: Study Details | Comprehensive HHT Outcomes Registry of the United States (CHORUS) | ClinicalTrials.gov

Genetic Alliance	**Institutional Review Board (IRB)**

STUDY SITES

Approximately 15 HHT Centers of Excellence across the U.S. will participate. Sites actively recruiting are listed below:

Site	Contact	Contact Email	Contact Phone
Augusta University	Melissa James, RN	mejames@augusta.edu	706-721-5599
The Cleveland Clinic	JoAnne Baran, RN	baranj2@ccf.org	216-645-1372
Mayo Clinic	Greg Schwichtenberg	schwichtenberg.greg@mayo.edu	507-255-1245

	Sue Ann P. Donlinger	Donlinger.sueann@mayo.edu	507-284-9259
Massachusetts General Hospital	Ceanna Kalaria, BA	ckalaria@harvard.mgh.edu	617-726-3520
New York Presbyterian/ Columbia University Irving Medical Center	Kelsey Weddig, RN, BSN	kw3149@cumc.columbia.edu	212-305-7470
Oregon Health and Science University	Lori Russell, RN	watsonlo@ohsu.edu	503-494-7226
University of Alabama at Birmingham	Tyler Prisoc	jtprisoc@uabmc.edu	205-934-9146
	Josie Harris, BSN, RN	jcrespo@uabmc.edu	205-996-9647
University of California, Los Angeles	Niloofar (Lily) Sheshebor	nsheshebor@mednet.ucla.edu	310-562-9752
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University of California, San Francisco	Bridget Kilbride	bridget.kilbride@ucsf.edu	415-514-6221
University of Colorado, Denver	Johan Allingmon	johan.allingmon@cuanschutz.edu	303-724-6052
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	Isaac Elysee, MS, CGC	Isaac.Elysee@Pennmedicine.upenn.edu	215-614-0833
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	Kristine Kempf, RN	kempf@wustl.edu	314-273-8131
Yale University	Katharine Henderson, MS, CGC	katharine.henderson@yale.edu	203-737-1427