# The American Thrombosis & Hemostasis Network Current Projects

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# Collecting data, connecting people – and making progress

ATHN and our affiliated centers are committed to improving the quality of life for people with blood disorders. In everything we do, we strive to facilitate continuity of care, promote collaboration, maintain confidentiality, and conserve resources through a common technology infrastructure.

Together, we're building the ATHNdataset, a safe, secure national data set that is being used to improve care and support vital research. This unique community resource is helping researchers gain a better understanding of hematologic disorders from clinical, social, and economic perspectives.

Through the ATHNdataset and various ATHN research projects, we're making real progress.

# An Overview of Current Projects

The American Thrombosis and Hemostasis Network (ATHN) is a nonprofit organization dedicated to supporting and expanding opportunities for the network of ATHN-affiliated treatment centers. We're dedicated to serving the hematologic disorders community by helping to improve the lives of people affected by non-neoplastic blood disorders.

We're using technology to secure data, advance knowledge, and transform care. We partner with centers—our ATHN Affiliates—across the country on a number of innovative projects. And we collaborate with researchers to facilitate many of these various projects and studies, with each study drawing from ATHN's secure data resources to gain a better understanding of the issues affecting people with hematologic disorders. Each project is helping to make a difference.

# A Natural History Cohort Study of the Safety, Effectiveness, and Practice of Treatment in People with Non-Neoplastic Hematologic Disorders

ATHN Transcends is a cohort study to determine the safety, effectiveness, and practice of therapies used in the treatment of participants with congenital or acquired non-neoplastic blood disorders and connective tissue disorders with bleeding tendency. Participants are followed longitudinally for at least 15 years from the time of enrollment. The study consists of 7 cohorts: Hemophilia, Von Willebrand Disease, Thrombosis/Thrombophilia, Non-Neoplastic Hematologic Conditions, Bleeding Not Otherwise Specified (NOS), Rare Bleeding Disorders, and Congenital Platelet Disorders. Each cohort may have various study "arms" with their own principal investigators.

**Principal Investigators:** Michael Recht, MD, PhD, MBA, American Thrombosis and Hemostasis Network and Lynn Malec, MD, MSc, Versiti Blood Research Institute

# ATHN Transcends: Hemophilia Natural History Arm

(Formerly ATHN 7: A Natural History Cohort Study of the Safety, Effectiveness, and Practice of Treatment for People with Hemophilia)

The Hemophilia Natural History Arm of ATHN Transcends, formerly ATHN 7: Hemophilia Natural History Study, is a longitudinal, observational study to determine the safety of newer treatment alternatives such as non-factor molecules, as well as older therapies such as factor concentrate and bypassing agents. It also aims to evaluate the effectiveness of these various hemophilia treatment products when used for prevention and for surgery.

**Principal Investigators:** Tyler Buckner, MD, MSc, Hemophilia and Thrombosis Center, University of Colorado Anschutz Medical Campus and Michael Recht, MD, PhD, The Hemophilia Center at Oregon Health & Science University

**Status:** Open for enrollment as part of ATHN Transcends for specific centers

# ATHN Transcends: Hemophilia Gene Therapy Outcomes Arm

(Formerly ATHN 14: A Longitudinal Cohort Study of the Safety and Effectiveness of Gene Therapy for People with Hemophilia)

The ATHN Transcends Hemophilia Gene Therapy Outcomes Arm, formerly known as ATHN 14: Hemophilia Gene Outcomes Study, is a pragmatic study designed to understand the outcomes of gene therapy with real-world practices across a wide range of products, including the safety of adeno-associated viral vector or lentiviral vecto-mediated factor VIII and factor IX therapies when used for participants with hemophilia.

**Status:** Study arm is scheduled to open in Q4 2021

**Principal Investigators:** Janice M. Staber, MD; Iowa Hemophilia and Thrombosis Center, University of Iowa Stead Family Children's Hospital; and Ulrike M. Reiss, MD, Hemophilia Treatment Center, St. Jude Children's Research Hospital

ATHN 8

## U.S. Cohort Study of Previously Untreated Patients (PUPs) with Congenital Hemophilia

ATHN 8: PUPs Study is the first national study focused on previously untreated patients that will provide a platform for ongoing clinical studies to improve the outcomes of this vulnerable population. Its aim is to determine the percentage of PUPs with confirmed inhibitors within the first 50 exposure days of clotting factor therapies and risk factors associated with inhibitor development.

**Principal Investigators:** Shannon Carpenter, MD, MS, Children's Mercy Hospital and Courtney Thornburg, MD, MS, Rady Children's Hospital

This project is in collaboration with Versiti Blood Center of Wisconsin and the Centers for Disease Control and Prevention.

**Status:** Currently open for enrollment

ATHN 9

# A Natural History Cohort Study of Safety, Effectiveness, and Practice of Treatment for People with Severe Von Willebrand Disease (VWD)

Von Willebrand disease (VWD) is the most common inherited bleeding disorder in humans, caused by missing or defective von Willebrand factor (VWF), a clotting protein. ATHN 9: Severe VWD Natural History Study is aimed at assessing the safety and effectiveness of various VWF treatment regimens in both pediatric and adult patients with clinically severe VWD.

**Principal Investigators:** Robert Sidonio, MD, Aflac Cancer and Blood Disorders Center, Hemophilia of Georgia Center for Bleeding and Clotting Disorders and Angela Weyand, MD, C.S. Mott Children's Hospital

**Status:** Currently open to enrollment

ATHN 10

# Leveraging the ATHNdataset to Document the State of Rare Coagulation Disorders in the United States

ATHN 10: Rare Coagulation Disorders Project offers free genetic testing to patients who have opted in to have their data contributed to the ATHNdataset. The goal of this project is to enhance the genotypic and phenotypic data on the population of rare coagulation disorder patients receiving care within the ATHN-affiliated network.

**Principal Investigators:** Amy Shapiro, MD, Indiana Hemophilia & Thrombosis Center and Diane Nugent, MD, Center for Comprehensive Care and Diagnosis of Inherited Blood Disorders

**Status:** Open for enrollment

# ATHN 11

## An Observational, Cohort Study of Long-Term Outcomes of Orthotopic Liver Transplantation in People with Hemophilia

The primary objective of ATHN 11: Liver Transplantation Outcomes Study is to examine the impact of sustained increase in factor VIII and factor IX levels after liver transplantation on quality of life in adults with hemophilia A and B compared to patients who have not undergone liver transplantation. This multi-center, observational, cohort study will use questionnaires to measure quality of life of participants based on levels of pain, health outcomes, work and leisure activity, and overall mental and physical health.

**Principal Investigator:** Margaret Ragni, MD, Hemophilia Center of Western Pennsylvania

**Status:** Open for enrollment

ATHN 12

# ATHNdataset Hereditary Antithrombin Deficiency (HAD) Pilot Project

Hereditary antithrombin (AT) deficiency (HAD) is a major risk factor for the development of venous thromboembolism (VTE) in adults and children. ATHN 12: HAD Pilot Project is collecting data on patients diagnosed with HAD across the U.S. to better understand this rare condition. Data gathered will be used to inform the development of guidelines regarding proper time of screening for HAD, type of AT therapy, and adequate use of AT concentrate.

**Principal Investigator:** Maria DeSancho, MD, Weill Cornell Medicine,
New York Presbyterian Hospital

**Status:** Open

ATHN 15

# Characterizing the Real-World Use of Direct Oral Anticoagulants (DOACs) in Pediatric Thrombosis Patients

ATHN 15: DOAC Use in Pediatric Thrombosis Patients is a national project aimed at characterizing the real-world use of direct oral anticoagulant (DOAC) products in pediatric patients. Designed to be a resource for clinicians, this project includes data on pediatric patients under 21 years of age who have received a DOAC since January 1, 2015 for the treatment of an acute venous thromboembolism (VTE) episode or to prevent a thrombosis recurrence.

**Principal Investigators:** Jennifer Davila, MD, Montefiore Medical Center and Fernando Corrales-Medina, MD, Hemophilia Treatment Center at University of Miami

**Status:** Closed for enrollment

ATHN 16

# ATHN 16 Safety of SEVENFACT® for the Treatment of Bleeding Events in Patients with Hemophilia A or B with Inhibitors

ATHN 16: SEVENFACT® for Bleeding Events in Hemophilia with Inhibitors aims to evaluate the safety of the FDA-approved drug SEVENFACT when used to treat bleeding episodes in participants with hemophilia A or B with inhibitors who are being treated either with or without prophylactic agents. The study is a phase IV multi-center, U.S.-centric, open-label, safety study and is the first interventional clinical trial that ATHN has sponsored.

**Primary Investigators:** Tammuella Chrisentery-Singleton, MD, Mississippi Center for Advanced Medicine (MCAM) and Louisiana Center for Advanced Medicine (LCAM) and Mark T. Reding, MD, University of Minnesota Medical Center

**Status:** Open for enrollment

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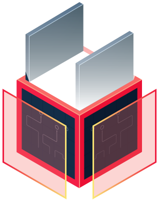
# Conducting Research with ATHN

ATHN proudly collaborates with researchers to facilitate a variety of studies through our network of federally-funded treatment centers and ATHN affiliates, ATHN offers data and statistical services, as well as study implementation and support services, scientific leadership, and funding opportunities. For research projects ATHN is the sponsor. The synergy created by working through ATHN helps reduce time, costs, and hassles of project start-up.

ATHN has research capabilities that can be mixed and matched to meet your specific study needs, including:

→ Electronic data capture
→ Data management services
→ Study management
→ Communication services
→ Administrative services
→ Publication support

**Visit What We Do for Researchers at athn.org to learn more.**



**If you are a provider interested in working with ATHN on one of our current projects, or if you are a researcher interested in proposing a project, please email us at**

**support@athn.org.**