

# Hematology/Hemophilia/ Thrombosis Protocol List

July 2021

Hemophilia and thrombosis treatment protocols			
Study	Clinical trial name	Phase/type	Age
<b>Pfizer Apixaban Ph 4</b>	A Randomized, Open-label, Active Controlled, Safety and Extrapolated Efficacy Study in Pediatric Subjects Requiring Anticoagulation for the Treatment of a Venous Thromboembolic Event <a href="https://clinicaltrials.gov/ct2/show/NCT02464969">https://clinicaltrials.gov/ct2/show/NCT02464969</a>	III/IV	<2 yr
<b>VWDMin</b>	Prospective, Randomized, Crossover Trial Comparing Recombinant von Willebrand Factor (rVWF) vs. Tranexamic Acid (TA) to Minimize Menorrhagia in Women with Type 1 von Willebrand Disease: The VWD Minimize Study <a href="https://clinicaltrials.gov/ct2/show/NCT02606045">https://clinicaltrials.gov/ct2/show/NCT02606045</a>	III	18 to 45 yr
Hemophilia and thrombosis biology protocols			
Study	Clinical trial name	Phase/type	Age
<b>PPG Zimmerman vWD</b>	Zimmerman Program on the Biology of VWD	Non-therapeutic	>2 yr
<b>Characterization FVIII Inhibitors</b>	Characterization of Factor VIII Inhibitors	Non-therapeutic	All ages
<b>Platelet Physiology</b>	In vitro Analysis of Platelet Physiology and Pharmacology [open specimen collection for basic science studies]	Non-therapeutic	All ages
<b>Platelet Contraction</b>	In vitro Analysis of Platelet Contraction and Physiology	Non-therapeutic	All ages
<b>MOSH in Hemophilia</b>	An Observational Study of the Natural History of Outcomes in Hemophilics Undergoing Major Orthopedic Surgery <a href="https://clinicaltrials.gov/ct2/show/NCT04618237">https://clinicaltrials.gov/ct2/show/NCT04618237</a>	Non-therapeutic	All ages
<b>ATHN9</b>	A Natural History Cohort Study of the Safety, Effectiveness, and Practice of Treatment for People with Severe Von Willebrand Disease (VWD) <a href="https://clinicaltrials.gov/ct2/show/NCT03853486">https://clinicaltrials.gov/ct2/show/NCT03853486</a>	Non-therapeutic	All ages (males and females)
<b>ATHN 8 PUPs</b>	US Cohort Study of Previously Untreated Patients (PUPs) with Congenital Hemophilia (PUPs Matter) <a href="https://clinicaltrials.gov/ct2/show/NCT03818529">https://clinicaltrials.gov/ct2/show/NCT03818529</a>	Non-therapeutic	All ages
<b>FVIII Pratt</b>	Design of Less Immunogenic Factor VIII Proteins	Non-therapeutic	All ages

<b>Hem Biorepository</b>	Emory University/Children's Healthcare of Atlanta (CHOA) Hemostasis and Thrombosis Biorepository <a href="https://clinicaltrials.gov/ct2/show/NCT04398628">https://clinicaltrials.gov/ct2/show/NCT04398628</a>	Non-therapeutic	Any age (includes undiagnosed family members)
<b>Hemophilia and thrombosis supportive treatment and non-therapeutic protocols</b>			
<b>Hem MOTIVATE</b>	MODern Treatment of Inhibitor - PositiVe PATiEnts with Haemophilia A – An International Observational Study (MOTIVATE) <a href="https://clinicaltrials.gov/ct2/show/NCT04023019">https://clinicaltrials.gov/ct2/show/NCT04023019</a>	Non-therapeutic	Males any age
<b>Hematology treatment protocols</b>			
<b>Study</b>	<b>Clinical trial name</b>	<b>Phase/type</b>	<b>Age</b>
<b>APL2-PNH-209</b>	APL2-PNH-209, An Open-label, Single-arm, Phase 2 Study to Evaluate the Safety, Pharmacokinetics, and Biologic Activity of Pegcetacoplan in Pediatric Patients with Paroxysmal Nocturnal Hemoglobinuria	II	≥12 to ≤17 yr
<b>ICON3 ETB115JUS33T</b>	A Phase 3 Study of Eltrombopag vs. Standard First-Line Management for Newly Diagnosed Immune Thrombocytopenia (ITP) in Children <a href="https://clinicaltrials.gov/ct2/show/NCT03939637">https://clinicaltrials.gov/ct2/show/NCT03939637</a>	III	1 to <18 yr
<b>Gen Hem AVA-Ped-301</b>	A Phase 3b, Multi-center, Randomized, Double-blind, Placebo-controlled, Parallel-group Trial with an Open-label Extension Phase to Evaluate the Efficacy and Safety of Avatrombopag for the Treatment of Thrombocytopenia in Pediatric Subjects with Immune Thrombocytopenia for ≥6 Months <a href="https://clinicaltrials.gov/ct2/show/NCT04516967">https://clinicaltrials.gov/ct2/show/NCT04516967</a>	IIIb	≥6 mo
<b>Hematology biology protocols</b>			
<b>Study</b>	<b>Clinical trial name</b>	<b>Phase/type</b>	<b>Age</b>
<b>Hem Biorepository</b>	Emory University/Children's Healthcare of Atlanta (CHOA) Hemostasis and Thrombosis Biorepository <a href="https://clinicaltrials.gov/ct2/show/NCT04398628">https://clinicaltrials.gov/ct2/show/NCT04398628</a>	Non-therapeutic	Any age (includes undiagnosed family members)
<b>Hemoglobin Disorder Biorep</b>	Characterizing the Complications Associated with Therapeutic Blood Transfusions for Hemoglobinopathies	Non-therapeutic	Any age
<b>Hematology non-therapeutic protocols</b>			
<b>Study</b>	<b>Clinical trial name</b>	<b>Phase/type</b>	<b>Age</b>
<b>Agios PKD Registry</b>	AG348-C-008, Pyruvate Kinase Deficiency Global Longitudinal Registry <a href="https://clinicaltrials.gov/ct2/show/NCT03481738">https://clinicaltrials.gov/ct2/show/NCT03481738</a>	Registry	Any age
<b>PARC ITP</b>	Pediatric and Adult Intercontinental Registry on Chronic Idiopathic Thrombocytopenia Purpura (worldwide database on chronic ITP followed by side studies)	Registry	≥2 mo

<b>NAPAAC: PRO in BMF</b>	Treatment Related Patient Reported Outcomes in Bone Marrow Failure Syndromes including Severe Pediatric Aplastic Anemia	Non-therapeutic	5 to 17.9 yr
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