

Hematology/Hemophilia/ Thrombosis Protocol List

March 2021

Hemophilia and thrombosis treatment protocols			
Study	Clinical trial name	Phase/type	Age
Pfizer Apixaban Ph 4	A Randomized, Open-label, Active Controlled, Safety and Extrapolated Efficacy Study in Pediatric Subjects Requiring Anticoagulation for the Treatment of a Venous Thromboembolic Event https://clinicaltrials.gov/ct2/show/NCT02464969	III/IV	<2 yr
Explorer 7	Explorer 7: Efficacy and Safety of Concizumab Prophylaxis in Patients with Haemophilia A or B with Inhibitors https://clinicaltrials.gov/ct2/show/NCT04083781	IIIa	≥12 yr
VWDMin	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 https://clinicaltrials.gov/ct2/show/NCT02606045	III	18 to 45 yr
WIL-31	Clinical Study to Investigate the Efficacy and Safety of <i>WILATE</i> during Prophylaxis in Previously Treated Patients with Von Willebrand Disease (VWD) https://clinicaltrials.gov/ct2/show/NCT04052698	III	≥6 yr
Hemophilia and thrombosis biology protocols			
Study	Clinical trial name	Phase/type	Age
Bioverativ 18-11141	Single Cell Sequencing to Study Immunogenicity and Immune Tolerance of Factor VIII (Exploratory Study)	Non-therapeutic	All ages
PPG Zimmerman vWD	Zimmerman Program on the Biology of VWD	Non-therapeutic	>2 yr
Characteriza-tion FVIII Inhibitors	Characterization of Factor VIII Inhibitors	Non-therapeutic	All ages
Platelet Physiology	In vitro Analysis of Platelet Physiology and Pharmacology [open specimen collection for basic science studies]	Non-therapeutic	All ages
Platelet Contraction	In vitro Analysis of Platelet Contraction and Physiology	Non-therapeutic	All ages
MOSH in Hemophilia	An Observational Study of the Natural History of Outcomes in Hemophilics Undergoing Major Orthopedic Surgery https://clinicaltrials.gov/ct2/show/NCT04618237	Non-therapeutic	All ages

ATHN9	A Natural History Cohort Study of the Safety, Effectiveness, and Practice of Treatment for People with Severe Von Willebrand Disease (VWD) https://clinicaltrials.gov/ct2/show/NCT03853486	Non-therapeutic	All ages (males and females)
ATHENA1	ATHENA1: Characterizing the Impact and Treatment of Reproductive Tract Bleeding on Women and Post-menarchal Girls with Bleeding Disorders	Non-therapeutic	10 to 65 yr
ATHN 8 PUPs	US Cohort Study of Previously Untreated Patients (PUPs) with Congenital Hemophilia (PUPs Matter) https://clinicaltrials.gov/ct2/show/NCT03818529	Non-therapeutic	All ages
FVIII Pratt	Design of Less Immunogenic Factor VIII Proteins	Non-therapeutic	All ages
Hem Biorepository	Emory University/Children's Healthcare of Atlanta (CHOA) Hemostasis and Thrombosis Biorepository https://clinicaltrials.gov/ct2/show/NCT04398628	Non-therapeutic	Any age (includes undiagnosed family members)
Hemophilia and thrombosis supportive treatment and non-therapeutic protocols			
WIL-29	A Prospective, Multicentre, Non-Interventional Study Evaluating the Bleeding Incidence in Patients with Von Willebrand Disease Undergoing On-Demand Treatment (WIL-29) https://clinicaltrials.gov/ct2/show/NCT04053699	Non-therapeutic	≥5.5 yr
QOL in Women	Quality of Life in Adolescents and Adult Females with Congenital Bleeding Disorders and Heavy Menstrual Bleeding	Non-therapeutic	9 to 55 yr
Hematology treatment protocols			
Study	Clinical trial name	Phase/type	Age
APL2-PNH-209	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
Agios AG348-C-011	AG348-C-011, An Open-Label, Multi-Center, Extension Study of AG-348 in Adult Subjects with Pyruvate Kinase Deficiency Previously Enrolled in AG-348 Studies https://clinicaltrials.gov/ct2/show/NCT03853798	III	any age
ALXN1210-PNH-304	Protocol ALXN1210-PNH-304: A Phase 3, Open-label Study of ALXN1210 in Children and Adolescents with Paroxysmal Nocturnal Hemoglobinuria https://clinicaltrials.gov/ct2/show/NCT03406507	III	<18 yr
ICON3 ETB115JUS33T	A Phase 3 Study of Eltrombopag vs. Standard First-Line Management for Newly Diagnosed Immune Thrombocytopenia (ITP) in Children https://clinicaltrials.gov/ct2/show/NCT03939637	III	1 to <18 yr
Hematology biology protocols			
Study	Clinical trial name	Phase/type	Age
Hem Biorepository	Emory University/Children's Healthcare of Atlanta (CHOA) Hemostasis and Thrombosis Biorepository https://clinicaltrials.gov/ct2/show/NCT04398628	Non-therapeutic	Any age (includes undiagnosed family members)

Hemoglobin Disorder Biorep	Characterizing the Complications Associated with Therapeutic Blood Transfusions for Hemoglobinopathies	Non-therapeutic	Any age
Hematology non-therapeutic protocols			
Study	Clinical trial name	Phase/type	Age
Agios PKD Registry	AG348-C-008, Pyruvate Kinase Deficiency Global Longitudinal Registry https://clinicaltrials.gov/ct2/show/NCT03481738	Registry	Any age
PARC ITP	Pediatric and Adult Intercontinental Registry on Chronic Idiopathic Thrombocytopenia Purpura (worldwide database on chronic ITP followed by side studies)	Registry	≥2 mo
NAPAAC: PRO in BMF	Treatment Related Patient Reported Outcomes in Bone Marrow Failure Syndromes including Severe Pediatric Aplastic Anemia	Non-therapeutic	5 to 17.9 yr