Hematology/Hemophilia/ Thrombosis

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
Betrixaban	A Phase 1, Open-label, Single-dose, Non-randomized Study to Evaluate Pharmacokinetics, Pharmacodynamics, and Safety of Betrixaban in Pediatric Patients	I	12 to <18 yr (Part I)
Kids-DOTT	Prospective Multi-Center Evaluation of the Duration of Therapy for Thrombosis in Children (the “Kids-DOTT Trial) https://clinicaltrials.gov/ct2/show/NCT00687882	III	Birth to <21 yr

Hematology treatment protocols			
AMGABP959 Dahlia	A Randomized, Double-blind, Active-controlled Phase 3 Study Evaluating the Efficacy and Safety of ABP 959 Compared with Eculizumab in Adult Subjects with Paroxysmal Nocturnal Hemoglobinuria (PNH) https://clinicaltrials.gov/ct2/show/NCT03818607	III	≥18 yr
ACTIVATE	Clinical Study Protocol AG348-C-006: A Phase 3, Randomized, Double-blind, Placebo-controlled Study to Evaluate the Efficacy and Safety of AG-348 in Not Regularly Transfused Adult Subjects with Pyruvate Kinase Deficiency https://clinicaltrials.gov/ct2/show/NCT03548220	III	≥18 yr
ACTIVATE-T	Clinical Study Protocol AG348-C-007: An Open-label Study to Evaluate the Efficacy and Safety of AG-348 in Regularly Transfused Adult Subjects with Pyruvate Kinase (PK) Deficiency https://clinicaltrials.gov/ct2/show/NCT03559699	III	≥18 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
ETB115E2201	A Phase II, Open-label, Non-controlled, Intra-patient Dose-escalation Study to Characterize the Pharmacokinetics after Oral Administration of Eltrombopag in Pediatric Patients with Refractory, Relapsed or Treatment Naïve Severe Aplastic Anemia or Recurrent Aplastic Anemia https://clinicaltrials.gov/ct2/show/NCT03025698	II	1 to <18 yr
ARQ 092-103	ARQ 092-103: A Phase 1/2 Study of ARQ 092 (Miransertib) in Subjects with PIK3CA-related Overgrowth Spectrum and Proteus Syndrome https://clinicaltrials.gov/ct2/show/NCT03094832	I/II	≥2 to ≤30 yr