

Sickle Cell/Thalassemia/ Non-Thrombosis Protocol List

March 2021

Sickle cell, thalassemia and non-thrombosis treatment protocols			
Study	Clinical trial name	Phase/type	Age
SCD GBT440-041	GBT440-041: An Open-label, Expanded Access Protocol for Pediatric Patients with Sickle Cell Disease Who Have No Alternative Treatment Options https://clinicaltrials.gov/ct2/show/NCT04724421	OLE	≥4 to ≤11 yr
SCD GBT440-038	GBT440-038: An Open-label Extension Study of Voxelotor Administered Orally to Pediatric Participants with Sickle Cell Disease Who Have Participated in Voxelotor Clinical Trials https://clinicaltrials.gov/ct2/show/NCT04188509	OLE	≥4 to ≤18 yr
SCD GBT440-039	A Phase 4, Multicenter, Open-label Study to Evaluate the Treatment Effect of Voxelotor on Physical Activity in Adolescents and Adults with Sickle Cell Disease https://clinicaltrials.gov/ct2/show/NCT04400487	IV	≥12 yr
SCD STAND	A Phase III, Multicenter, Randomized, Double-blind Study to Assess Efficacy and Safety of Two Doses of Crizanlizumab versus placebo, with or without Hydroxyurea/Hydroxycarbamide Therapy, in Adolescent and Adult Sickle Cell Disease Patients with Vaso-Occlusive Crises (STAND) https://clinicaltrials.gov/ct2/show/NCT03814746	III	≥12 yr
SCD HOPS Pilot	Hydroxyurea Optimization through Precision Study (HOPS) https://clinicaltrials.gov/ct2/show/NCT03789591	III	≥6 mo to ≤21 yr
SCD GBT440-032 HOPE Kids 2	GBT440-032: A Phase 3, Randomized, Double-Blind, Placebo-Controlled Study of Voxelotor (GBT440) in Pediatric Participants with Sickle Cell Disease (HOPE Kids 2) https://clinicaltrials.gov/ct2/show/NCT03036813	III	2 to <15 yr
SCD GBT440-007	GBT440-007: A Phase 2a, Open-label, Single and Multiple Dose Study to Evaluate the Pharmacokinetics, Safety, Tolerability and Treatment Effect of GBT440 in Pediatric Participants with Sickle Cell Disease https://clinicaltrials.gov/ct2/show/NCT02850406	IIa	≤17 yr but depends on arm
SCD IMR-SCD-301	A Phase 2b Study to Evaluate the Safety and Efficacy of IMR-687 in Subjects with Sickle Cell Disease https://clinicaltrials.gov/ct2/show/NCT04474314	IIb	≥18 to ≤65 yr

SCD CSEG101B2201	CSEG101B2201: A Phase 2, Multicenter, Open-Label Study to Assess Appropriate Dosing and to Evaluate Safety of Crizanlizumab, with or without Hydroxyurea/Hydroxycarbamide, in Sequential, Descending Age Groups of Pediatric Sickle Cell Disease Patients with Vaso-Occlusive Crisis https://www.clinicaltrials.gov/ct2/show/NCT03474965	II	2 to <18 yr
SCD FORMA FT-4202- HVS-101	A Randomized, Placebo-controlled, Double Blind, Single Ascending and Multiple Ascending Dose Study to Assess the Safety, Pharmacokinetics and Pharmacodynamics of FT-4202 in Healthy Volunteers and Sickle Cell Disease Patients https://clinicaltrials.gov/ct2/show/NCT03815695	I	SCD Patients ≥12 yr
Sickle cell, thalassemia and non-thrombosis biology protocols			
Study	Clinical trial name	Phase/type	Age
SCD High Throughput	High Throughput Screening of Compounds for Sickle Cell Disease	Biology	≥3 yr
Sickle cell and thalassemia supportive treatment and non-therapeutic protocols			
Study	Clinical trial name	Phase/type	Age
SCD SPRINTS	SPRINTS: Sickle Cell Pro-Inflammatory Response to Interval Training Study https://clinicaltrials.gov/ct2/show/NCT03653676	Non-therapeutic	≥10 to ≤21 yr
SCD DISPLACE Part 3	Dissemination and Implementation of Stroke Prevention Looking at the Care Environment (DISPLACE) https://clinicaltrials.gov/ct2/show/NCT04173026	Non-therapeutic	Patients probably <24 yr; caregivers any age
SCD Experience CBT	A Qualitative Study of Patient and Family Experience with Chronic Blood Transfusion for Sickle Cell Disease	Non-therapeutic	12-18 yr
SCD CASCADE	Complement Alternate Pathway Activation in Sickle Cell Disease (CASCADE Study)	Non-therapeutic	≤21 yr
SCD iCanCope	iCanCope with Sickle Cell https://clinicaltrials.gov/ct2/show/NCT03201874	Non-therapeutic	12-18 yr
SCD Back2Life	Building Adaptive Coping and Knowledge to Improve Daily Life (Back2Life): A Family-Centered Approach to Improving Chronic Pain Management in Youth with Sickle Cell Disease https://clinicaltrials.gov/ct2/show/NCT04602728	Non-therapeutic	10-18 yr
SCD Back2Life CBT	Building Adaptive Coping and Knowledge to Improve Daily Life (Back2Life): A Pilot Feasibility Clinical Trial for Youth with Chronic Sickle Cell Pain	Non-therapeutic	10-18 yr and parents
SCD CBF	Microvascular Cerebral Blood Flow in Sickle Cell Disease	Non-therapeutic	2-18 yr

SCD MobileDOT	Pilot Study of Feasibility and Acceptability of HU Buddy [Patient Centered Comprehensive Medication Adherence Management System to Improve Effectiveness of Disease Modifying Therapy with Hydroxyurea in Patients with Sickle Cell Disease]	Non-therapeutic	>2 to 65 yr
Trans Chronic SCD Pain	A Prospective Study of Transition to Chronic Pain in Sickle Cell Disease	Non-therapeutic	12 to 16 yr
Biophys Charac Hem Disorders	Study on Sickle Cell and Other Hematological Disorders	Non-therapeutic	Any age
RBC Allo-immunization	Knowledge of Red Blood Cell (RBC) Alloimmunization in Children, Adolescents, and Adults with Hemoglobinopathies	Non-therapeutic	Any age
SCD ST3P-UP-001	A Comparative Effectiveness Study of Peer Mentoring [PM] versus Structured Transition Education Based Intervention [STE] for the Management of Care Transitions in Emerging Adults with Sickle Cell Disease (SCD) https://clinicaltrials.gov/ct2/show/NCT03593395	Non-therapeutic	16 to 25 yr
SCD Qualif ePain Diary	Qualification of A Multi-Dimensional Electronic Pain Diary in Children, Adolescents and Adults with Sickle Cell Disease	Non-therapeutic	≥4 yr