

# Sickle Cell/Thalassemia/ Non-Thrombosis Protocol List

July 2021

Sickle cell, thalassemia and non-thrombosis treatment protocols			
Study	Clinical trial name	Phase/type	Age
<b>SCD GBT440-041</b>	GBT440-041: An Open-label, Expanded Access Protocol for Pediatric Patients with Sickle Cell Disease Who Have No Alternative Treatment Options <a href="https://clinicaltrials.gov/ct2/show/NCT04724421">https://clinicaltrials.gov/ct2/show/NCT04724421</a>	OLE	≥4 to ≤11 yr
<b>SCD GBT440-038</b>	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 <a href="https://clinicaltrials.gov/ct2/show/NCT04188509">https://clinicaltrials.gov/ct2/show/NCT04188509</a>	OLE	≥4 to ≤18 yr
<b>SCD GBT440-039</b>	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 <a href="https://clinicaltrials.gov/ct2/show/NCT04400487">https://clinicaltrials.gov/ct2/show/NCT04400487</a>	IV	≥12 yr
<b>SCD STAND</b>	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) <a href="https://clinicaltrials.gov/ct2/show/NCT03814746">https://clinicaltrials.gov/ct2/show/NCT03814746</a>	III	≥12 yr
<b>SCD HOPS Pilot</b>	Hydroxyurea Optimization through Precision Study (HOPS) <a href="https://clinicaltrials.gov/ct2/show/NCT03789591">https://clinicaltrials.gov/ct2/show/NCT03789591</a>	III	≥6 mo to ≤21 yr
<b>SCD GBT440-032 HOPE Kids 2</b>	GBT440-032: A Phase 3, Randomized, Double-Blind, Placebo-Controlled Study of Voxelotor (GBT440) in Pediatric Participants with Sickle Cell Disease (HOPE Kids 2) <a href="https://clinicaltrials.gov/ct2/show/NCT03036813">https://clinicaltrials.gov/ct2/show/NCT03036813</a>	III	2 to <15 yr
<b>SCD GBT440-007</b>	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 <a href="https://clinicaltrials.gov/ct2/show/NCT02850406">https://clinicaltrials.gov/ct2/show/NCT02850406</a>	IIa	≤17 yr but depends on arm
<b>SCD IMR-SCD-301</b>	A Phase 2b Study to Evaluate the Safety and Efficacy of IMR-687 in Subjects with Sickle Cell Disease <a href="https://clinicaltrials.gov/ct2/show/NCT04474314">https://clinicaltrials.gov/ct2/show/NCT04474314</a>	IIb	≥18 to ≤65 yr

<b>SCD CSEG101B2201</b>	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 <a href="https://www.clinicaltrials.gov/ct2/show/NCT03474965">https://www.clinicaltrials.gov/ct2/show/NCT03474965</a>	II	2 to <18 yr
<b>SCD FORMA FT-4202- HVS-101</b>	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 <a href="https://clinicaltrials.gov/ct2/show/NCT03815695">https://clinicaltrials.gov/ct2/show/NCT03815695</a>	I	SCD Patients ≥12 yr
<b>GBT021601-012</b>	An Inpatient Single Dose and Multiple Ascending Dose Study to Evaluate the Pharmacokinetics, Safety, Tolerability, and Pharmacodynamics of GBT021601, a Hemoglobin S Polymerization Inhibitor, in Participants with Sickle Cell Disease (SCD)	Ib	18 to ≤60 yr
<b>SCD PRAISE</b>	An Adaptive, Randomized, Placebo-controlled, Double-blind, Multi-center Study of Oral FT-4202, a Pyruvate Kinase Activator in Patients with Sickle Cell Disease (PRAISE)	II/III	12 to ≤65 yr
<b>Sickle cell, thalassemia and non-thrombosis biology protocols</b>			
<b>Study</b>	<b>Clinical trial name</b>	<b>Phase/type</b>	<b>Age</b>
<b>SCD High Throughput</b>	High Throughput Screening of Compounds for Sickle Cell Disease	Biology	≥3 yr
<b>Sickle cell and thalassemia supportive treatment and non-therapeutic protocols</b>			
<b>Study</b>	<b>Clinical trial name</b>	<b>Phase/type</b>	<b>Age</b>
<b>SCD SPRINTS</b>	SPRINTS: Sickle Cell Pro-Inflammatory Response to Interval Training Study <a href="https://clinicaltrials.gov/ct2/show/NCT03653676">https://clinicaltrials.gov/ct2/show/NCT03653676</a>	Non-therapeutic	≥10 to ≤21 yr
<b>SCD DISPLACE Part 3</b>	Dissemination and Implementation of Stroke Prevention Looking at the Care Environment (DISPLACE) <a href="https://clinicaltrials.gov/ct2/show/NCT04173026">https://clinicaltrials.gov/ct2/show/NCT04173026</a>	Non-therapeutic	Patients probably <24 yr; caregivers any age
<b>SCD Experience CBT</b>	A Qualitative Study of Patient and Family Experience with Chronic Blood Transfusion for Sickle Cell Disease	Non-therapeutic	12-18 yr
<b>SCD CASCADE</b>	Complement Alternate Pathway Activation in Sickle Cell Disease (CASCADE Study)	Non-therapeutic	≤21 yr
<b>SCD iCanCope</b>	iCan Cope with Sickle Cell <a href="https://clinicaltrials.gov/ct2/show/NCT03201874">https://clinicaltrials.gov/ct2/show/NCT03201874</a>	Non-therapeutic	12-18 yr
<b>SCD Back2Life</b>	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 <a href="https://clinicaltrials.gov/ct2/show/NCT04602728">https://clinicaltrials.gov/ct2/show/NCT04602728</a>	Non-therapeutic	10-18 yr

<b>SCD Back2Life CBT</b>	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
<b>SCD CBF</b>	Microvascular Cerebral Blood Flow in Sickle Cell Disease	Non-therapeutic	2-18 yr
<b>Trans Chronic SCD Pain</b>	A Prospective Study of Transition to Chronic Pain in Sickle Cell Disease	Non-therapeutic	12 to 16 yr
<b>Biophys Charac Hem Disorders</b>	Study on Sickle Cell and Other Hematological Disorders	Non-therapeutic	Any age
<b>RBC Allo-immunization</b>	Knowledge of Red Blood Cell (RBC) Alloimmunization in Children, Adolescents, and Adults with Hemoglobinopathies	Non-therapeutic	Any age
<b>SCD ST3P-UP-001</b>	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) <a href="https://clinicaltrials.gov/ct2/show/NCT03593395">https://clinicaltrials.gov/ct2/show/NCT03593395</a>	Non-therapeutic	16 to 25 yr
<b>SCD Qualif ePain Diary</b>	Qualification of A Multi-Dimensional Electronic Pain Diary in Children, Adolescents and Adults with Sickle Cell Disease	Non-therapeutic	≥4 yr
<b>SCD VoxICON</b>	Interviews with Sickle Cell Disease (SCD) Patients to Understand Voxelotor Treatment Experience	Non-therapeutic	any age