

Sickle Cell Disease and General Hematology

| Sickle cell, thalassemia and non-thrombosis treatment protocols | | | |
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| Study | Clinical trial name | Phase/type | Age |
| 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 (Am 5) https://clinicaltrials.gov/ct2/show/NCT02850406 | IIa | ≤17 yr but depends on arm |
| 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 Arginine Ph II | Phase 2 Randomized Control Trial of Arginine Therapy for Pediatric Sickle Cell Disease Pain https://www.clinicaltrials.gov/ct2/show/NCT02536170 | II | 3 to 21 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 (vers 10-23-18) https://www.clinicaltrials.gov/ct2/show/NCT03474965 | II | 2 to <18 yr |

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| SCD SPARTAN | CSEG101AUS05: A Prospective Phase II, Open-Label, Single-arm, Multicenter, Study to Assess Efficacy and Safety of SEG101 (crizanlizumab), in Sickle Cell Disease Patients with Priapism (SPARTAN) https://clinicaltrials.gov/ct2/show/NCT03938454?term=CSEG101AUS05&rank=1 | II | ≥16 yr |
| SCD Arginine | Arginine Therapy for the Treatment of Pain in Children with Sickle Cell Disease https://www.clinicaltrials.gov/ct2/show/NCT02447874 | Pilot | 7 to 21 yr |
| Sickle cell, thalassemia and non-thrombosis biology protocols | | | |
| 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 | | | |
| SCD DISPLACE Part 2 | Dissemination and Implementation of Stroke Prevention Looking at the Care Environment (DISPLACE) (CHOA vers 7-13-18) | Non-therapeutic | Patients probably <24 yr; caregivers any age |

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| SCD Yoga | Acceptability, Feasibility and Safety of a Yoga Program for the Treatment of Chronic Pain in Sickle Cell Disease (vers 5-14-18) | Non-therapeutic | ≥12 yr |
| SCD Experience CBT | A Qualitative Study of Patient and Family Experience with Chronic Blood Transfusion for Sickle Cell Disease (vers 7-10-18) | Non-therapeutic | 12-18 yr |
| SCD CASCADE | Complement Alternate Pathway Activation in Sickle Cell Disease (CASCADE Study) (vers 3-15-19) | Non-therapeutic | ≤21 yr |
| SCD iCanCope | iCanCope with Sickle Cell (vers 6-21-19) | 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 (vers 9-24-18) | Non-therapeutic | 10-18 yr |
| SCD CBF | Microvascular Cerebral Blood Flow in Sickle Cell Disease (vers 5-6-19) | Non-therapeutic | 2-18 yr |
| SCD Decision Aid | Comparative Effectiveness of a Decision Aid for Therapeutic Options in Sickle Cell Disease (vers 4-17-18) | Non-therapeutic | [cohort dependent] |
| SCD Resilience Adol | Exploring Resilience and Adaptation in Adolescents with Sickle Cell Disease (vers 6-15-15) | Non-therapeutic | 12-18 yr and parents |
| 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 | Non-therapeutic | >2 to 65 yr |

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| | Hydroxyurea in Patients with Sickle Cell Disease] | | |
| 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) | Non-therapeutic | 16 to 25 yr |