## Pediatric Clinical Trials Office Research (PCTO) Bone Marrow Transplant

Sponsor ID	Protocol Title	Basic Eligibility	Status	
Sickle Cell Disease				
STAR MSD	Early HLA Matched Sibling Hematopoietic Stem Cell Transplantation for Children with Sickle Cell Disease	Age 2-13; HLA identical sibling donor <13yo, PSC enrolled	Active, Enrolling	
BMT CTN 1507	Reduced Intensity Conditioning for Haploidentical Bone Marrow Transplantation in Patients with Symptomatic Sickle Cell Disease	Age 5-45; HLA-matched sibling donor	Active, Peds cohort closes 10/10/22	
STAR PSC	Project Sickle Cure	Planned HSCT for SCD.	Active, Enrolling	
Prophylaxis				
STAR ASCENT	Acute GVHD Suppression using Costimulation Blockage to Expand Non-malignant Transplant (ASCENT)	SCD age 3 to <21, other dz age 0-<21; Must have unrelated adult donor 7 or 8/8 match	Active No slots available	
Kadmon Corp <u>KDO25</u> <u>Rockstar</u>	A Phase 2, Randomized, Multicenter Study to Evaluate the Efficacy and Safety of KD025 in Subjects with Chronic Graft Versus Host Disease (cGVHD) After At Least 2 Prior Lines of Systemic Therapy	Age >12, 2-5 prior lines of tx, persistent manifestations, weight ≥40kg	Active, Enrolling	
AlloVir <u>ALVR105</u>	Phase 2, Multicenter, Randomized, Double-Blind, Placebo-Controlled Study to Assess the Safety and Efficacy of Viralym-M Compared to Placebo for the Prevention and Treatment of AdV, BKV, CMV, EBV, HHV-6, and JCV Infection &/or Disease, in High-Risk Patients After Allogeneic Hematopoietic Cell Transplant	HSCT within 15-42 days, no viral infx or asymptomatic, 1 suitably matched cell line	Active, Enrolling	
SCIDS				

PIDTC 6907	Severe Combined Immune Deficiency: Prospective and Longitudinal Study of Genotypes, Management and Outcomes	SCID spectrum disorders w/ intention to treat with HCT	Active, Enrolling	
PBMTC NMD 1801 <u>CSIDE</u>	A randomized trial of low versus moderate exposure busulfan for infants with severe combined immunodeficiency (SCID) receiving TCR $\alpha\beta$ +/CD19+ depleted transplantation: A Phase II study by the PIDTC and PBMTC	Age up to 2	Active, Enrolling	
CAR-T				
COG IST AALL1721	CTL019G2201J/AALL1721 - A phase II trial of tisagenlecleucel in first-line high-risk (HR) pediatric and young adult patients with B-cell acute lymphoblastic leukemia (B-ALL) who are MRD positive at the (EOC) therapy	Age 1-25, CD19+ Exclude: Ph+, prior TKI, hypodiploid, genetic syndromes	Active Enrollment HOLD	
Novartis CTL019	Managed Access Program (MAP) to provide access to CTL019, for recurrent or refractory ALL or large B-cell lymphoma patients with out of specification leukapheresis product and/or manufactured tisagenlecleucel out of specification for commercial release	Up to age 25, out of spec and repeat leukapheresis not feasible	Active Enrolling	
Quality of Life				
CIBMTR 17-SIBS	Identifying Predictors of Poor Health-Related Quality-of-Life among Pediatric Hematopoietic Stem Cell Donors	Donor/recipient/sibling age 5-17; parent	Active Enrolling	