Pediatric Clinical Trials Office Research (PCTO)

Bone Marrow Transplant

Sponsor ID	Protocol Title	Basic Eligibility	Status
Treatment			
KDO25 Rockstar	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
EndRAD	Eliminating Total Body Irradiation (TBI) for NGS-MRD Negative Children, Adolescents, and Young Adults With **B-ALL**	Age 1-25, obs vs trmt based on MRD; T-ALL/MPAL eligible for obs.	Active, Enrolling
ASCT2031	Mismatched Related Donor Versus Matched Unrelated Donor Stem Cell Transplantation for Children, Adolescents, and Young Adults With **Acute Leukemia or Myelodysplastic Syndrome**	6months -22 y old; ALL, AML or MDS in CR; No MSD available	Active, Enrolling
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
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
AlloVir ALVR105	Phase 2/3, Multicenter, Randomized, Double-Blind, Placebo-Controlled Study to Assess the Safety and Efficacy of **Posoleucel (ALVR105, Viralym-M) Compared to Placebo** for the Prevention of AdV, BKV, CMV, EBV, HHV-6, and JCV Infection and/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
PIDTC 6909	Neurodevelopmental Outcomes Following Treatment for Severe Combined Immunodeficiency (SCID)	6-16yrs, previously enrolled on 6901/6902	Active, Enrolling
PBMTC NMD 1801 CSIDE	A randomized trial of low versus moderate exposure busulfan for infants with severe combined immunodeficiency (SCID) receiving TCRαβ+/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
Umbilical Cord Blood			
CIBMTR 10-CBA	A multicenter access and distribution protocol for unlicensed cryopreserved cord blood units (CBUs) for transplantation in pediatric and adult patients with hematologic malignancies and other indications	Any age	Active, Enrolling