Bone Marrow Transplant

March 2021

Aflac Cancer & Blood Disorders Center
CHILDREN'S HEALTHCARE OF ATLANTA

BMT for malignancy treatment protocols			
Study	**Clinical trial name**	**Phase/type**	**Age**
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 https://clinicaltrials.gov/ct2/show/NCT01351545	Access protocol	Any age
NCBP Cord Blood	A multicenter safety study of unlicensed, investigational cryopreserved cord blood units (CBUs) manufactured by the National Cord Blood Program (NCBP) and provided for unrelated hematopoietic stem cell transplantation of pediatric and adult patients (Protocol 6637-01) https://clinicaltrials.gov/ct2/show/NCT01656603	Access protocol	Any age
Novartis CART FU	Protocol No. CCTL019A2205B: Long Term Follow-up of Patients Exposed to Lentiviral-Based CD19 directed CART Cell Therapy https://clinicaltrials.gov/ct2/show/NCT02445222	NA	*Any age (received anti-CD19 directed CART therapy)*
PBMTC Onc1701 EndRad	A Phase II Pilot Trial to Estimate Survival after a Non-total Body Irradiation (TBI) based Conditioning Regimen in Patients Diagnosed with B-acute Lymphoblastic Leukemia (ALL) who are Pre-allogeneic Hematopoietic Cell Transplantation (HCT) Next-generation Sequence (NGS) Minimal Residual Disease (MRD) Negative https://clinicaltrials.gov/ct2/show/NCT03509961	II	≥1 to ≤25 yr
Novartis Cassiopeia (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 Minimal Residual Disease (MRD) Positive at the End of Consolidation (EOC) Therapy https://clinicaltrials.gov/ct2/show/NCT03876769	II	1 to 25 yr
Novartis CART ELIANA	Protocol CCTL019B2202: A Phase II, Single Arm, Multicenter Trial to Determine the Efficacy and Safety of CTL019 in Pediatric Patients with Relapsed and Refractory B-cell Acute Lymphoblastic Leukemia https://clinicaltrials.gov/ct2/show/NCT02435849	II	≥3 to ≤21 yr
Novartis BIANCA	Protocol CCTL019C2202: A Phase II, Single Arm, Multicenter Open Label Trial to Determine the Safety and Efficacy of Tisagenlecleucel in Pediatric Patients with Relapsed or Refractory Mature B-cell non-Hodgkin Lymphoma (NHL) (BIANCA) https://clinicaltrials.gov/ct2/show/NCT03610724	II	<18 yr

HEAD START IV	HEAD START 4 PROTOCOL: Newly Diagnosed Children (Less Than 10 Years Old) With Medulloblastoma And Other Central Nervous System Embryonal Tumors. Clinical and Molecular Risk-Tailored Intensive and Compressed Induction Chemotherapy Followed By Consolidation With Randomization To Either Single-Cycle Or To Three Tandem Cycles Of Marrow-Ablative Chemotherapy With Autologous Hematopoietic Progenitor Cell Rescue https://clinicaltrials.gov/ct2/show/NCT02875314	IV	<10 yr
BMT for non-malignancy treatment protocols			
Study	**Clinical trial name**	**Phase/type**	**Age**
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 https://clinicaltrials.gov/ct2/show/NCT01351545	Access protocol	Any age
NCBP Cord Blood	A multicenter safety study of unlicensed, investigational cryopreserved cord blood units (CBUs) manufactured by the National Cord Blood Program (NCBP) and provided for unrelated hematopoietic stem cell transplantation of pediatric and adult patients (Protocol 6637-01) https://clinicaltrials.gov/ct2/show/NCT01656603	Access protocol	Any age
BMT NI-0501-09	An Open-label, Single Arm, Multicenter Study to Broaden Access to Emapalumab, an AntiInterferon Gamma (Anti-IFNγ) Monoclonal Antibody, and to Assess its Efficacy, Safety, Impact on Quality of Life, and Long-term Outcome in Pediatric Patients with Primary Hemophagocytic Lymphohistiocytosis	Access protocol	≤18 yr
COG ANHL1522	ANHL1522, A Pilot Study of Rituximab (RTX) and Third Party Latent Membrane Protein (LMP)-specific Cytotoxic T-Lymphocytes (LMP-TC, IND # 17068) in Pediatric Solid Organ Recipients (SOT) with EBV-Positive CD20-Positive Post-Transplant Lymphoproliferative Disease (PTLD) https://clinicaltrials.gov/ct2/show/NCT02900976	Pilot	<30 yr
SCID-X1	Phase I/II Trial of Lentiviral Gene Transfer for SCID-X1 with Low Dose Targeted Busulfan Conditioning https://clinicaltrials.gov/ct2/show/NCT03311503	I/II	<5 yr
ST-400-01	A Phase 1/2, Open-label, Single-arm Study to Assess the Safety, Tolerability, and Efficacy of ST-400 Autologous Hematopoietic Stem Cell Transplant for Treatment of Transfusion-dependent β-thalassemia (TDT) https://clinicaltrials.gov/ct2/show/NCT03432364	I/II	≥18 yr
BIV003	A Phase 1/2, Open-Label, Multicenter, Single-Arm Study to Assess the Safety, Tolerability, and Efficacy of BIVV003 for Autologous Hematopoietic Stem Cell Transplantation in Patients with Severe Sickle Cell Disease	I/II	18 to 35 yr
STAR ASCENT	**A**cute GVHD **S**uppression using **C**ostimulation Blockade to **E**xpand **N**on-malignant **T**ransplant (ASCENT) https://clinicaltrials.gov/ct2/show/NCT03924401	II	3 to 20 yr (SCD) ≤20 yr (other dis)

TRANSFORM	Transplantation using Reduced Intensity Approach for Patients with Sickle Cell Disease from Mismatched Family Donors of Bone Marrow (TRANSFORM Study) https://clinicaltrials.gov/ct2/show/NCT02757885	II	≥15 yr (or younger only if pubertal)
BMT STAR MSD	HLA Matched Related Hematopoietic Stem Cell Transplantation for Children with Less Severe Sickle Cell Disease: a Sickle Transplant Alliance for Research (STAR) Trial https://clinicaltrials.gov/ct2/show/NCT04018937	II	≥2 to <10 yr
BMT PBMTC 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 Primary Immune Deficiency Treatment Consortium (PIDTC) and Pediatric Blood and Marrow Transplant Consortium (PBMTC) PIDTC "CSIDE" Protocol (Conditioning SCID Infants Diagnosed Early) PBMTC NMD 1801 https://clinicaltrials/gov/ct2/show/NCT03619551	II	0 to 2 yr
BMT Atara ATA129-EBV-302	ATA129-EBV-302: Multicenter, Open Label, Phase 3 Study of Tabelecleucel for Solid Organ or Allogeneic Hematopoietic Cell Transplant Subjects with Epstein-Barr Virus-Associated Post-Transplant Lymphoproliferative Disease after Failure of Rituximab or Rituximab and Chemotherapy (ALLELE Study) https://clinicaltrials.gov/ct2/show/NCT03394365	III	Any age
BMT AB2Bio	Multicenter, Double-blind, Placebo-controlled, Randomized Withdrawal Trial with Tadekinig alfa (r-hIL-18BP) in Patients with IL-18 driven Monogenic Autoinflammatory Conditions: NLRC4 Mutation and XIAP Deficiency https://clinicaltrials.gov/ct2/show/NCT03113760	III	≤17 yr
BMT supportive treatment and non-therapeutic protocols			
Study	**Clinical trial name**	**Phase/type**	**Age**
BMT CTN 1702	Clinical Transplant-Related Long-term Outcomes of Alternative Donor Allogeneic Transplantation https://clinicaltrials.gov/ct2/show/NCT03904134	Non-therapeutic	Any age
17-SIBS	Identifying Predictors of Poor Health-Related Quality-of-Life among Pediatric Hematopoietic Stem Cell Donors	Non-therapeutic	≥5 to ≤7 yr
COG ALTE05N1	Umbrella Long-Term Follow-Up Protocol https://clinicaltrials.gov/ct2/show/NCT00736749	Non-therapeutic	All ages
STAR Retrospective Registry	A Multi-center Retrospective Registry of Children with Sickle Cell Disease following Hematopoietic C Transplantation: A Sickle Transplant Alliance for Research (STAR) Project	Registry	≤25 yr at time of HCT for SCD

STELLAR	Sickle Cell Transplant Evaluation of Long term and Late-effects Registry (STELLAR)	Registry	any age
SCD Pain PROs	Study of Pain, Patient Reported Outcomes (PROs) and Experimental Pain Sensitivity in Children with SCD Prior to and Following Bone Marrow Transplantation for Sickle Cell Disease	Non-therapeutic	≥8 yr
MIPLATE	Clinical Effectiveness of Conventional Versus Mirasol-treated Apheresis Platelets in Patients with Hypoproliferative Thrombocytopenia (MIPLATE) https://clinicaltrials.gov/ct2/show/NCT02964325	Non-therapeutic	>10 kg
SCD Acceptability and Usability Tool	Acceptability and Usability of a Brief Decision Support Tool for Bone Marrow Transplant for Sickle Cell Disease	Non-Therapeutic	Healthcare providers who treat SCD
PSC	Project Sickle Cure	Non-therapeutic	<25 yr
PREDICT	PRospective Pilot Study of Noninvasive Imaging and Blood Biomarkers of Endothelial Dysfunction In Children with Thrombotic Microangiopathy after Hematopoietic Cellular Therapy (PREDICT)	Non-therapeutic	<2 yr
BMT biology protocols			
Study	**Clinical trial name**	**Phase/type**	**Age**
NMDP-CIBMTR	The National Marrow Donor Program (NMDP) and Center for International Blood and Marrow Transplant Research (CIBMTR) Protocols for a Research Database and Sample Repository for Hematopoietic Stem Cell Transplantation and Marrow Toxic Injuries	Biology	All ages
BMT RDCRN PIDTC 6901	A Prospective Natural History Study of Diagnosis, Treatment and Outcomes of Children with SCID Disorders (RDCRN PIDTC #6901)	Biology	All ages
BMT RDCRN PIDTC 6902	A Retrospective and Cross-sectional Analysis of Patients Treated for SCID since January 1, 1968	Biology	All ages
BMT RDCRN PIDTC 6903	Analysis of Patients Treated for Chronic Granulomatous Disease Since January 1, 1995	Biology	All ages
BMT HELP SCD	HLA Antibody Evaluation and Platelet Transfusions (HELP) in Transplant for Sickle Cell Disease	Biology	Any age
GvHD treatment protocols			
Study	**Clinical trial name**	**Phase/type**	**Age**

Autologous MSCs for GvHD	A Phase I Study of Mesenchymal Stromal Cells for the Treatment of Acute and Chronic Graft versus Host Disease	I	>12 yr
Itacitinib Mt. Sinai	Itacitinib Monotherapy for Low Risk Graft-Vs-Host Disease	II	≥12 yr
BMT Vedo-3035	A Randomized, Double-Blind, Placebo-Controlled, Multicenter Study to Evaluate the Efficacy and Safety of Vedolizumab in the Prophylaxis of Intestinal Acute Graft-Versus-Host Disease in Subjects Undergoing Allogeneic Hematopoietic Stem Cell Transplantation	III	≥12 yr