# Bone Marrow Transplant

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

| BMT for malignancy treatment protocols | | | |
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| **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 | 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) | Access protocol | Any age |
| **Novartis CART FU** | Protocol No. CCTL019A2205B: Long Term Follow-up of Patients Exposed to Lentiviral-<br>Based CD19 directed CART Cell Therapy<br>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<br>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<br>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<br>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)<br>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 | IV | <10 yr |

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| | https://clinicaltrials.gov/ct2/show/NCT02875314 | | |
| **BMT for non-malignancy treatment protocols** | | | |
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| **Abatacept NMD** | Abatacept for Post-Transplant Immune Suppression in Children and Adolescents Receiving Allogeneic Hematopoietic Stem Cell Transplants for Non-Malignant Diseases https://clinicaltrials.gov/ct2/show/NCT01917708 | Pilot | 1 to 21 yr |
| **STAR Aba GvHD SCD** | Abatacept for Graft Versus Host Disease Prophylaxis after Hematopoietic Stem Cell Transplantation for Pediatric Sickle Cell Disease: A Sickle Transplant Alliance for Research Trial https://clinicaltrials.gov/ct2/show/NCT02867800 | Pilot | 3 to <21 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 |
| **HGB-206** | Clinical Study Protocol HGB-206: A Phase 1 Study Evaluating Gene Therapy by Transplantation of Autologous CD34+ Stem Cells Transduced Ex Vivo with the LentiGlobin BB305 Lentiviral Vector in Subjects with Severe Sickle Cell Disease https://clinicaltrials.gov/ct2/show/NCT02140554 | I | ≥18 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) | II | ≥15 yr (or younger |

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| | https://clinicaltrials.gov/ct2/show/NCT02757885 | | only if pubertal) |
| **BMT CTN 1503 STRIDE 2** | A Study to Compare Bone Marrow Transplantation to Standard Care in Adolescents and Young Adults with Severe Sickle Cell Disease<br>https://clinicaltrials.gov/ct2/show/NCT0276646 | II | ≥15 yr |
| **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<br>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:<br>A Phase II Study by the Primary Immune Deficiency Treatment Consortium (PIDTC) and Pediatric Blood and Marrow Transplant Consortium (PBMTC)<br>PIDTC "CSIDE" Protocol<br>(Conditioning SCID Infants Diagnosed Early)<br>PBMTC NMD 1801<br>https://clinicaltrials.gov/ct2/show/NCT03619551 | II | 0 to 2 yr |
| **BMT Magenta IMD-001** | Protocol IMD-001: A Phase 2, Single-arm, Open-label Study to Evaluate the Safety and Efficacy of MGTA-456 in Patients with Inherited Metabolic Disorders (IMD) Undergoing Hematopoietic Stem Cell (HSC) Transplantation (HSCT)<br>https://clinicaltrials.gov/ct2/show/NCT03406962 | II | >6 mo to <16 yr |
| **BMT JAZZ 15-007** | Clinical Study Protocol 15-007: A Phase 3, Randomized, Adaptive Study Comparing the Efficacy and Safety of Defibrotide vs Best Supportive Care in the Prevention of Hepatic Veno-Occlusive Disease in Adult and Pediatric Patients Undergoing Hematopoietic Stem Cell Transplantation<br>https://clinicaltrials.gov/ct2/show/NCT02851407 | III | >1 mo |
| **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<br>Mutation and XIAP Deficiency<br>https://clinicaltrials.gov/ct2/show/NCT03113760 | III | ≤17 yr |
| **BMT supportive treatment and non-therapeutic protocols** | | | |
| **BMT Rifaximin** | Rifaximin for Infection Prophylaxis in Hematopoietic Stem Cell Transplantation<br>https://clinicaltrials.gov/ct2/show/NCT03529825 | Pilot | ≥2 to ≤21 yr |
| **ACES** | Antiviral Cellular Therapy for Enhancing T-cell Reconstitution Before or After Hematopoietic Stem Cell Transplantation (ACES) PBMTC SUP1701 | I/II | All ages |
| **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<br>https://clinicaltrials.gov/ct2/show/NCT00736749 | Non-therapeutic | All ages |

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| **PBMTC SUP1601** | Pathogen Identification in Pediatric Hematopoietic Stem Cell Transplant Patients with Suspected Lower Respiratory Tract Infection https://clinicaltrials.gov/ct2/show/NCT02926612 | Non-therapeutic | ≤21 yr |
| **STAR Retrospective Registry** | A Multi-center Retrospective Registry of Children with Sickle Cell Disease following Hematopoietic Cell 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 Sensit in Children with SCD Prior to and Following Bone Marrow Transplantation for Sic Cell Disease | Non-therapeutic | ≥8 yr |
| **HU Buddy Post-HSCT** | HU-Buddy: A Medication Adherence Application for Post-hematopoietic Stem Cell Transplant Patients with and without Chronic GVHD | Non-therapeutic | ≥10 to ≤21 yr |

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| **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 |
| **BMT biology protocols** | | | |
| **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 | | | |
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| **Autologous MSCs for GvHD** | A Phase I Study of Mesenchymal Stromal Cells for the Treatment of Acute and Chronic Graft versus Host Disease https://clinicaltrials.gov/ct2/show/NCT02359929 | I | >12 yr |