Appendix Table E1. List of ongoing studies

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| **Research Gap** | **Study** | **Study Objective (group by this)** | **Research Design** | **Sample size;**  **Years;**  **Location;**  **Followup** | **Treatment(s)** | **Outcomes** | **Status** |
| **EWING SARCOMA** | Phase 3, Open Label, Multi-centre, Randomized Controlled International Study in Ewing Sarcoma; University Hospital Münster, NCT00987636 | To examine whether high dose chemotherapy using busulfan-melphalan with autologous stem cell reinfusion, compared with standard chemotherapy, improves event-free survival in patients with localized Ewing sarcoma and unfavorable histological response or tumor volume>200ml.  To examine whether the addition of high dose chemotherapy using treosulfan-melphalan followed by autologous stem cell reinfusion to eight cycles of standard adjuvant chemotherapy, compared with eight cycles of standard adjuvant chemotherapy alone, improves event-free survival in patients with primary disseminated disease. | Efficacy Study | 1383  10/2009-3/2018  Germany  FU: 6.5, 8.5 years | HSCT compared with standard chemotherapy | Event free survival  Overall survival  Safety and toxicity  Quality of life | This study is currently recruiting participants. |

| Appendix Table E1. List of ongoing studies (continued) | | | | | | | |
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| **Research Gap** | **Study** | **Study Objective (group by this)** | **Research Design** | **Sample size;**  **Years;**  **Location;**  **Followup** | **Treatment(s)** | **Outcomes** | **Status** |
| **EWING SARCOMA** | Combination Chemotherapy With or Without Peripheral Stem Cell Transplantation, Radiation Therapy, and/or Surgery in Treating Patients With Ewing's Sarcoma; University Hospitals, Leicester, NCT00020566 | To study different combination chemotherapy regimens to see how well they work when given with or without peripheral stem cell transplantation, radiation therapy, and/or surgery in treating patients with Ewing's sarcoma. | Treatment Study | 1200  2/2001-12/2011  United Kingdom  Patients are followed every 3 months for 4 years, every 6 months for 1 year, and then periodically thereafter. | chemotherapy with or without peripheral stem cell transplantation, radiation and/or surgery | Event free survival  Overall survival | This study has been suspended. |
| **WILMS TUMOR** | Chemotherapy Followed by Surgery and Radiation Therapy With or Without Stem Cell Transplant in Treating Patients With Relapsed or Refractory Wilms' Tumor or Clear Cell Sarcoma of the Kidney; Children's Cancer and Leukemia Group, United Kingdom, Ireland, NCT00025103 | To determine survival rates of patients with relapsed or refractory Wilms' tumor or clear cell sarcoma of the kidney treated with chemotherapy followed by surgical resection and adjuvant radiotherapy with or without autologous stem cell rescue.  To determine the efficacy and toxicity of these regimens in these patients.  To determine prognostic variables in patients treated with these regimens. | Treatment Study | 75  5/2001-11/2008  United Kingdom, Ireland  Patients are followed every 8 weeks for 1 year, every 12 weeks for 1 year, and then every 6 months thereafter. | Chemotherapy followed by surgery and radiation, with or without HSCT | Overall Survival  Toxicity  Prognostic variables | This study is ongoing, but not recruiting participants. |
| **RETINOBLASTOMA** | Combination Chemotherapy, Autologous Stem Cell Transplant, and/or Radiation Therapy in Treating Young Patients With Extraocular Retinoblastoma; Children's Oncology Group, United States, NCT00554788 | To study the side effects and how well giving combination chemotherapy together with autologous stem cell transplant and/or radiation therapy works in treating young patients with extraocular retinoblastoma. | Treatment Study | 60  2/2008-2/2014  United States  FU: Not reported | HDC, and HSCT and/or radiotherapy | Event-free survival  Response Rate  Toxicity | This study is currently recruiting participants. |
| **NEUROBLASTOMA** | Comparing Two Different Myeloablation Therapies in Treating Young Patients Who Are Undergoing a Stem Cell Transplant for High-Risk Neuroblastoma; Children's Oncology Group, United States, NCT00567567 | To compare EFS and tumor response of tandem HDC/HSCT and single HDC/HSCT | Treatment Study | 495  11/2007-3/2011  United States  FU: Not reported | tandem HDC/HSCT compared with single HDC/HSCT | Event-free survival  Response after induction therapy  Incidence rate of local recurrence  Duration of ≥ grade 3 neutropenia during course one  Duration of ≥ grade 3 thrombocytopenia during course one  Response rate after two courses of induction therapy | This study is currently recruiting participants. |
| **GERM CELL TUMORS** | High-dose Chemotherapy for Poor-prognosis Relapsed Germ-Cell Tumors; M.D. Anderson Cancer Center; Fred Hutchinson Cancer Research Center, Texas & Washington, NCT00936936 | To learn if bevacizumab, when given in combination with 2 cycles of high-dose chemotherapy, can help to control germ-cell tumors. | Safety/Efficacy Study | 25  6/2009- 6/2014  United States  FU: 2 years | 2 cycles of HDCT/SCT | 2-year Event-Free Survival | This study is currently recruiting participants. |
| **HIGH RISK SOLID TUMORS** | Tandem Peripheral Blood Stem Cell (PBSC) Rescue for High Risk Solid Tumors; Children's Memorial Hospital, Chicago, NCT00179816 | To study the feasibility and toxicity of tandem rescue with peripheral blood cells following HDC as consolidation in pediatric patients with high risk solid tumors | Safety/Efficacy Study | 12  4/1999- 9/2012  United States  FU: Annually until end of study | HDCT with tandem PBSCR | Toxicity  DFS  Stem cell dose to time of engraftment | This study is currently recruiting participants. |
| **HIGH RISK CNS TUMORS** | Stem Cell Transplant for High Risk Central Nervous System (CNS) Tumors; Children's Memorial Hospital, Chicago, NCT00179803 | To determine if a stem cell transplant in patients with newly diagnosed high risk CNS tumors (glioblastoma multiforme [GBM], high grade astrocytoma, pineoblastoma, rhabdoid tumor, supratentorial primitive neuroectodermal tumor [PNET]) increases overall survival. | Safety/Efficacy Study | 50  3/1998 – 1/2008  United States  FU: Not reported | HDCT with tandem SCR | OS  PFS  Adverse Events | This study is ongoing, but not recruiting participants. |
| **GLIOBLASTOMA MULTIFORME AND BRAIN STEM TUMORS** | Chemotherapy Followed by Bone Marrow or Peripheral Stem Cell Transplantation in Treating Patients With Glioblastoma Multiforme or Brain Stem Tumors; New York University School of Medicine, New York, NCT00002619 | To study the effectiveness of chemotherapy followed by autologous bone marrow or peripheral stem cell transplantation in treating patients with glioblastoma multiforme or brain stem tumors. | Phase II treatment study | 60 (ages 6 to 59 years)  9/1994 - End date not provided  United States  FU: Not reported | HDCT with autologous bone or peripheral stem cell transplant | OS  PFS  Time to progression /recurrence  Toxicity | This study is ongoing, but not recruiting participants. |
| **NEWLY DIAGNOSED/ RELAPSED SOLID TUMORS** | Busulfan, Melphalan, Topotecan Hydrochloride, and a Stem Cell Transplant in Treating Patients With Newly Diagnosed or Relapsed Solid Tumor; City of Hope Medical Center, California, NCT00638898 | To study how well giving busulfan, melphalan, and topotecan hydrochloride together with a stem cell transplant works in treating patients with newly diagnosed or relapsed solid tumor. | Efficacy Study | 25 patients  12/2006 – 4/2011  United States  FU: 1 year | HDCT with ASCR | OS  DFS  Engraftment incidence  Pharmacokinetics and pharmacodymanics of topotecan hydrochloride and busulfan | This study is currently recruiting participants. |
| **BRAIN AND CNS TUMORS, GERM CELL TUMORS** | High-Dose Chemotherapy With or Without Total-Body Irradiation Followed by Autologous Stem Cell Transplant in Treating Patients With Hematologic Cancer or Solid Tumors; Roswell Park Cancer Institute, New York, NCT00536601 | To study different high-dose chemotherapy regimens with or without total-body irradiation to compare how well they work when given before autologous stem cell transplant in treating patients with hematologic cancer or solid tumors. | Phase II/III Treatment Study | 530  6/2006 – 4/2024  United States  FU: 10 years | HDCT with ASCR with or without TBI | PFS  OS  Toxicity  Tumor response  Efficacy | This study is currently recruiting participants. |
| **GLIOBLASTOMA MULTIFORME AND GLIOSARCOMA** | Temozolomide, Carmustine, O6-Benzylguanine, Radiation Therapy, and an Autologous Stem Cell Transplant in Treating Patients With Newly Diagnosed Glioblastoma Multiforme or Gliosarcoma; Fred Hutchinson Cancer Research Center, Washington, NCT00669669 | To study the best dose of carmustine and temozolomide when given together with radiation therapy, carmustine, O6-benzylguanine, and an autologous stem cell transplant in treating patients with newly diagnosed glioblastoma multiforme or gliosarcoma. | Safety/Efficacy Study | 30  7/2006 – 2/2017  United States  FU: 15 years | Radiotherapy followed by HDCT and PBSCR | Toxicity  Development of replication competent retrovirus or leukemia  Tumor response  Time to progression  Chemoprotection (temozolomide dose)  Duration of response  Gene transfer efficiency and in vivo gene marking in peripheral blood and marrow | This study is currently recruiting participants. |
| **INHERITED METABOLIC DISEASE (adrenoleukodystrophy, metachromatic leukodystrophy, globoid cell leukodystrophy, Gaucher’s disease, fucosidosis, Wolman’s disease, Niemann-Pick disease, Batten disease, GM1 gangliosidosis, Tay-Sachs disease, and Sandhoff disease)** | Stem Cell Transplant for Inborn Errors of Metabolism; Masonic Cancer Center, University of Minnesota, Minnesota, NCT00176904 | To determine the safety and engraftment of donor hematopoietic cells using this conditioning regimen in patients undergoing a hematopoietic (blood forming) cell transplant for an inherited metabolic storage disease. | Efficacy Study | 134  1/1995 – 6/2010  United States  FU: 3 years | Surgery, chemotherapy and HSCT | OS  Change in neuropsychometric function  Toxicity  Engraftment  GVHD  Pharmacokinetic parameters | This study is ongoing, but not recruiting participants. |
| **TYPE 1 DIABETES MELLITUS** | Autologous Transplantation of Mesenchymal Stem Cells for Treatment of Patients With Onset of Type 1 Diabetes; Third Military Medical University, Chongqing, NCT01157403 | To study the safety and efficacy of autologous bone marrow mesenchymal stem cells in treatment of newly diagnosed patients with T1DM. | Safety/Efficacy Study | 80  7/2010 – 8/2012  China  FU: 2 years | Autologous bone marrow mesenchymal stem cell IV (2.5 x 106 cell/kg body weight) | C-peptide release test | This study is currently recruiting participants. |
| **TYPE 1 DIABETES MELLITUS** | Autologous Hematopoietic Stem Cell Transplantation for Early Onset Type 1 Diabetes; Shanghai Jiao Tong University School of Medicine, NCT00807651 | To evaluate whether stem cell transplantation is safe when chemotherapy and immunotherapy are used in combination and if it has immune resetting effect that may halt the immune attack to pancreas islets and thus preserve the body's own insulin production. | Safety/Efficacy Study | 30  2/2008 – 2/2013  China  FU: 3 years | HSC with cyclophosphamide and rabbit antithymocyte globulin IV | Exogenous insulin dose  Anti-GAD titers  C-peptide level  HbA1c level | This study is currently recruiting participants. |
| **TYPE 1 DIABETES MELLITUS** | Hematopoietic Stem Cell Transplantation in Type 1 Diabetes Mellitus; Hospital Universitario Dr. Jose E. Gonzalez, Mexico, NCT01121029 | To determine if autologous nonmyeloablative hematopoietic stem cell transplantation is able to induce prolonged and significant increases of C-peptide levels associated with absence of or reduction of daily insulin. | Safety/Efficacy Study | 15 (2-35 years of age)  5/2010 – 3/2011  Mexico  FU: 1 year | Nonmyeloablative conditioning followed by IV PBSCT | C-peptide level  HbA1c level | This study is currently recruiting participants. |
| **TYPE 1 DIABETES MELLITUS** | Safety and Efficacy Study of Autologous Stem Cell Transplantation for Early Onset Type I Diabetes Mellitus; University of Sao Paulo, Brazil, NCT00315133 | To evaluate the effect of inactivation of the immune system with chemotherapy and immunotherapy and infusion of bone marrow stem cells in early onset type 1 diabetes mellitus. | Safety/Efficacy Study | 24 (12 to 35 years of age)  12/2003 – 12/2012  Brazil  FU: 1 year | High dose immunosuppression with cyclophosphamide and rabbit antithymocyte globulin and PBSCT | Exogenous insulin dose  C-peptide level  Hemoglobin A1c  Quality of life measures  Anti-GAD titers  Immunogenic reconstitution parameters | This study is currently recruiting participants. |
| **TYPE 1 DIABETES MELLITUS** | Safety and Efficacy of Autologous Adipose-Derived Stem Cell Transplantation in Patients With Type 1 Diabetes; Adistem Ltd, Philippines, NCT00703599 | To study whether intravenous administration of autologous adipose stem cells is safe and beneficial in patients with type 1 diabetes. | Safety/Efficacy Study | 30  11/2007 – 12/2009  Philippines  FU: 4 years | Autologous adipose derived stem cell transplant | Lowering of insulin-dependence and hyperglycemic medication dosage  HbA1C level  C-peptide level  Quality of life measures  Kidney, liver and other hematological functioning parameters | This study is currently recruiting participants. |
| **SYSTEMIC LUPUS ERYTHEMATOSUS** | Cyclophosphamide and rATG/Rituximab in Patients With Systemic Lupus Erythematosus; Northwestern University, Chicago, NCT00278538 | To produce a normal immune system that will no longer attack body. The study purpose is to examine whether this treatment will result in improvement in the lupus disease. | Safety/Efficacy Study | 40 (16 to 60 years)  8/2005 – 8/2013  United States  FU: 5 years | AHSCT | OS of patients who achieve and maintain remission post transplant | This study is currently recruiting participants. |
| **SYSTEMIC LUPUS ERYTHEMATOSUS** | Mesenchymal Stem Cells Transplantation for Refractory Systemic Lupus Erythematosus (SLE); Nanjing Medical University, Jiangsu, China , NCT00698191 | To explore a new approach to treat patients with a medical condition known as systemic lupus erythematosus (SLE) who have been resistant to previous treatments using a new population of cells with capability to restore a normal immune system that will no longer attack the body. | Safety/Efficacy Study | 20 (15 to 70 years)  3/2007 – 12/2012  China  FU: 2 years | Cyclophosphamide administered before allogeneic bone marrow derived mesenchymal stem cell transplant (106 cells/kg body weight) | Systemic Lupus Erythematosus Disease Activity Index  Lupus serology (ANA, dsDNA, C3, C4)  Renal function (GFR, BUN, urinalysis)  Percentage of systemic T regulatory population | This study is currently recruiting participants. |
| **SYSTEMIC SCLEROSIS** | Allogeneic Mesenchymal Stem Cells Transplantation for Systemic Sclerosis (SSc); The Affiliated Nanjing Drum Tower Hospital of Nanjing University Medical School, Jiangsu, China, NCT00962923 | To explore safety and efficacy of allogeneic mesenchymal stem cells transplantation (MSCT) to treat patients with diagnosis of systemic sclerosis(SSc) who have been resistant to multiple standard treatments. | Treatment Study | 20 (15 to 65 years)  8/2009 – 12/2011  China  FU: Not Reported | Allogeneic mesenchymal stem cells will be infused intravenously (single dose, 10^6 cells/kg body weight). | mRSS score, HRQOL score, SF-36 score  Remission for organ function, VC, DLCO, PAP, serum albumin, serum creatinine, weight loss, 24h proteinuria  SSc Serology(ATA,ACA,ANA,anti-ssDNA,anti-dsDNA,IgM,IgG,and IgA, compliment C3 and C4  Change of peripheral blood B and T cells | This study is currently recruiting participants. |
| **SYSTEMIC SCLEROSIS** | Pilot Study of Total Body Irradiation in Combination With Cyclophosphamide, Anti-thymocyte Globulin, and Autologous CD34-Selected Peripheral Blood Stem Cell Transplantation in Children With Refractory Autoimmune Disorders; Fred Hutchinson Cancer Research Center, Seattle, Washington, NCT00010335 | To determine the safety and long term complication of TBI combined with PBSC for refractory autoimmune disorders. To determine the efficacy, engraftment rate, and reconstitution of immunity in these patients. | Safety/Efficacy Study | 20  11/2000 – 12/2020  United States  FU: 10 years | Stem cell transplantation along with irradiation and the drugs anti-thymocyte globulin, cyclophosphamide, and filgrastim | Mortality  Immune reconstruction  Engraftment  Efficacy  Late-effects | This study is ongoing, but not recruiting participants. |
| **CHRON’S DISEASE** | Autologous Stem Cell Transplantation for Crohn's Disease; Duke University, Miltenyi Biotec GmbH,Durham, North Carolina , NCT00692939 | To evaluate the safety and effectiveness of administering high-dose chemotherapy followed by infusion of autologous CD34-selected peripheral blood stem cells (PBSC) in pediatric and young adult patients with severe Crohn's disease. | Safety/Efficacy Study | 10 (5 to 25 years)  4/2005 – 12/2014  United States  FU: 5 years | High-dose immunotherapy followed by infusion of autologous CD34-selected peripheral blood stem cells | Evaluate the safety of administering high-dose immunotherapy followed by infusion of autologous CD34-selected peripheral blood stem cells  Pace of neutrophil and platelet recovery after the administration using ablative therapy and infusion of autologous CD34-selected PBSCs  Pace of reconstitution of immunity  Long-term complications, such as sterility, endocrinopathy, and growth failure  Immune function/profile  Describe the effects of this therapy on the clinical manifestations of Crohn’s Disease | This study is currently recruiting participants. |
| **CHRON’S DISEASE** | Hematopoietic Stem Cell Support in Patients With Severe Crohn's Disease. Northwestern University, Chicago, Illinois, NCT00278577 | To determine the safety and efficacy of immune ablation with stem cell support on the treatment of Crohn’s Disease. | Safety/Efficacy Study | 50 (up to 60 years)  4/2001 – 4/2011  United States  FU: 5 years | Immune Ablation and Hematopoietic Stem Cell Support | CDAI - If the index worsens by 50 points for more than 4 weeks, the disease will be considered progressive; if it improves by 70 points for more than four weeks, it will be considered improved; otherwise it will be considered stable | This study is ongoing, but not recruiting participants. |
| **SOCIAL SUPPORT (SARCOMA, NEUROBLASTOMA)** | A Web-Based Stem Cell Transplant Support System or Standard Care in Young Patients Undergoing Stem Cell Transplant and Their Families; Tufts Medical Center Cancer Center, United States, NCT00782145 | To study a web-based stem cell transplant support system to see how well it works compared with standard care in families of young patients undergoing a stem cell transplant. | Support Intervention Study | 200 children w/ accompanying parent undergoing HSCT (all transplant types)  6/2008 – 12/2010  United States  FU: 3, 6, 9, 12 mo testing after baseline | Web-based information and social support intervention with analysis of quality-of-life (Child Health Ratings Inventory) at baseline 3, 6, 9 and 12 months and Patient Health Questionnaire (PHQ-9) at baseline and 6 months. Parent completes measures for family and individual coping, social support, process of care and Internet use. | To evaluate the ability of a Web-based Hematopoietic Stem Cell Transplantation (HSCT-) Comprehensive Health Enhancement Support System (HSCT-CHESS) to mitigate the impact of a child's HSCT on the health-related quality of life, family functioning, knowledge, skills, and processes of care of the accompanying parent.  To explore the potential mechanisms of action of HSCT-CHESS in improving outcomes in these parents, in terms of parental activation, social support and/or coping skills.  To explore the impact of HSCT-CHESS on the health-related quality of life of the pediatric HSCT patient, as reported by the parent and child. | This study is currently recruiting participants. |
| **FUNGAL INFECTION** | Voriconazole Compared With Itraconazole in Preventing Fungal Infections in Patients Undergoing Allogeneic Hematopoietic Stem Cell Transplantation; Jonsson Comprehensive Cancer Center, California, NCT00079222 | To study voriconazole to see how well it works compared with itraconazole in preventing fungal infections in patients who are undergoing allogeneic hematopoietic stem cell transplantation. | Supportive care intervention | 150 (75 per arm)  1/2004 – End date not reported  United States  FU 180 days post transplant | Arm I: Beginning after allogeneic hematopoietic stem cell transplantation (AHSCT), patients receive voriconazole IV twice daily on days 1-14 and then orally\* twice daily on days 15-100.  Arm II: Beginning after AHSCT, patients receive itraconazole IV twice daily on days 1-2, once daily on days 3-14, and then orally\* twice daily on days 15-100. | Toxicity  Infection  Safety | This study is ongoing, but not recruiting participants. |
| **GENETIC RISK FOR SECONDARY MALIGNANCY** | Genetic Susceptibility and Risk of Second Cancers in Patients Who Have Undergone Stem Cell Transplant for Cancer; Vanderbilt-Ingram Cancer Center, Tennessee, NCT00949052 | To study genetic susceptibility and risk of second cancers in patients who have undergone stem cell transplant for cancer. | Observational study | 1000 (800 patients without secondary malignancy and 200 with secondary malignancy)  1/2009-1/2014  United States  FU: Not reported | Observational study | Genetic susceptibility to the carcinogenic effects of radiotherapy, tobacco, and UV light and risk of second malignant neoplasms.  Radiation sensitivity in B-cell lymphoblastoid cells  Allelic variants of genes involved in xenobiotics metabolism, DNA repair, and provision of nucleotide pool of patients with secondary malignancy compared with their first-degree relatives and patients without secondary malignancy  Role of potentially carcinogenic environmental exposures (tobacco and sun light) pre- and post-HSCT in the risk of secondary malignancy | This study is currently recruiting participants. |
| **LYSATE-PULSED DENDRITIC VACCINE FOR HIGH-RISK SOLID TUMORS** | A Pilot Study of Tumor Cell Vaccine for High-risk Solid Tumor Patients Following Stem Cell Transplantation; University of Michigan Cancer Center, Michigan, NCT00405327 | To investigate a tumor lysate-pulsed dendritic cell vaccine for immune augmentation after stem-cell transplantation for pediatric patients with high-risk solid tumors | Safety/Efficacy study | 30 patients (up to 30 years of age)  6/2006 -12/2013  United States  FU: 3 years | HSCT with tumor lysate-pulsed dendritic cell vaccine | Rate of immune response of this immunotherapy treatment  To correlate and characterize the immune response to the clinical response.  To define immunologic endpoints that can serve as surrogates of clinical response. | This study is ongoing, but not recruiting participants. |

**Abbreviations:** AHSCR: Autologous hematopoietic stem cell rescue; AHSCT: Autologous hematopoietic stem cell treatment; ASCR: Autologous stem cell rescue; CR: Complete remission; CSF: Cerebrospinal fluid; DOD: Dead of disease; EFS: Event-free survival; FU: Follow up; HDCT: High-dose chemotherapy; HSCT: Hematopoietic stem cell treatment;   
OS: Overall survival; PBSCT: Peripheral blood stem cell treatment; RBFS: Retinoblastoma free survival; RFS: Relapse free survival; SCT: Stem cell treatment; TRM: Treatment-related mortality