



Selected Protocols for the Treatment of Pediatric Cancer and Blood Disorders

This listing changes frequently.

It also does not include most of our Children's Oncology Group (COG) trials.

We are part of the COG Phase 1 Consortium – one of only 21 centers nationally, and the only one in New England. For more information about the trials we have available, please contact clinicaltrials@danafarberbostonchildrens.org

Treatment Area	Study Name	ID Number/ Phase	Site Investigator
EXPERIMENTAL THERAPEUTICS – early phase trials for patients with SOLID TUMORS, BRAIN TUMORS, AND/OR LYMPHOMA/LEUKEMIA			
Relapsed/Refractory Solid Tumors, CNS Tumors and Lymphoma	Phase 1 Trial of CUDC-907 in Children and Young Adults with Relapsed or Refractory Solid Tumors, CNS Tumors, or Lymphoma	16-371/I	Steven DuBois, MD, MS 617- 632-4450
Relapsed Refractory CNS and Solid Tumors	A Phase I Study of the EZH2 Inhibitor Tazemetostat in Pediatric Subjects with Relapsed or Refractory INI1-Negative Tumors or Synovial Sarcoma	15-467/I	Susan Chi, MD 617-632-4386
Relapsed Refractory CNS and Solid Tumors, including Neuroblastoma and Lymphoma	A Phase I Study of the Oral TRK Inhibitor LOXO-101 in Pediatric Patients with Advanced Solid or Primary Central Nervous System Tumors	16-094/I	Steven DuBois, MD, MS 617-632-4450
Relapsed Refractory CNS and Solid Tumors	A Phase 1/1b, Open-Label, Dose-Escalation and Expansion Study of Entrectinib (RXDX-101) in Children and Adolescents with Recurrent or Refractory Solid Tumors and Primary CNS Tumors	16-045/I-Ib	Suzanne Shusterman, MD 617-632-4901
Relapsed/Refractory Solid Tumors and CNS Tumors	A Phase 1, Open-Label, Dose-Escalation Study of Olaratumab as a Single Agent and in Combination with Doxorubicin, Vincristine/ Irinotecan, or High-Dose Ifosfamide in Pediatric Patients with Relapsed or Refractory Solid Tumors	16-196/I	Steven DuBois, MD, MS 617-632-4450
Relapsed Refractory Solid Tumors and Lymphoma	A Phase 1/2 Study Of Nivolumab (Ind# 124729) in Children, Adolescents, and Young Adults With Recurrent or Refractory Solid Tumors as a Single Agent and in Combination With Ipilimumab (ADVL1412)	16-722/I-II	Steven DuBois, MD, MS 617-632-4450
Relapsed/Refractory Medulloblastoma and Neuroblastoma	A Phase 1/2 Study of MK-1775 (AZD1775, IND# 121422) in Combination with Oral Irinotecan in Children, Adolescents, and Young Adults with Relapsed or Refractory Solid Tumors (ADVL1312)	16-447/I-II	Steven DuBois, MD, MS 617-632-4450
Relapsed Refractory CNS and Solid Tumors	A Phase I Study of Selinexor (KPT-330, IND 125052), a Selective XPO1 Inhibitor, in Recurrent and Refractory Pediatric Solid Tumors, Including CNS Tumors (ADVL1414)	15-718/I	Suzanne Shusterman, MD 617-632-4901

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EXPERIMENTAL THERAPEUTICS (continued)			
Relapsed Refractory Neuroectodermal Tumors, Rhabdomyosarcoma and/ or Other Solid Tumors	A Phase I Open Label, Dose Escalation Trial to Determine the MTD, Safety, PK and Efficacy of Afatinib Monotherapy in Children Aged 2 Years to <18 Years with Recurrent/Refractory Neuroectodermal Tumors, Rhabdomyosarcoma and/or Other Solid Tumors with Known ErbB Pathway Deregulation Regardless of Tumor Histology	15-315/I	Mark Kieran, MD, PhD 617-632-4386
Relapsed Refractory Solid Tumors and Lymphoma	An Early-Phase, Multicenter, Open-Label Study of the Safety and Pharmacokinetics of Anti-PD-L1 Antibody (MPDL3280A) in Pediatric and Young Adult Patients with Previously Treated Solid Tumors	15-308/I-II	Steven DuBois, MD, MS 617-632-4450
Relapsed/Refractory Low Grade Gliomas and other Solid Tumors	A Phase I/II Study of MEK162 for Children Low-Grade Gliomas and Other Ras/Raf/ERK Pathway Activated Tumors	15-368/I-II	Mark W. Kieran, MD, PhD 617-632-4386
Relapsed/Refractory Solid Tumors and Leukemia	Phase I Study of Carfilzomib in Combination With Cyclophosphamide and Etoposide for Children With Relapsed and Refractory Solid Tumors and Leukemias	15-588/I	Steven DuBois, MD, MS 617-632-4450
Relapsed/Refractory Solid Tumors and CNS Tumors	A Phase I/II, Multicenter, Open-Label, Dose-Escalation Study of the Safety and Pharmacokinetics of Cobimetinib in Pediatric and Young Adult Patients With Previously Treated Solid Tumors	15-524/I-II	Steven DuBois, MD, MS 617-632-4450
Relapsed Refractory CNS, Solid Tumors and Lymphoma	A Phase I/II Study of Pembrolizumab (MK-3475) in Children with advanced melanoma or a PD-L1 positive advanced, relapsed or refractory solid tumor or lymphoma	16-235/I-II	Steven DuBois, MD, MS 617-632-4450
Relapsed/Refractory Solid Tumors and CNS Tumors	A Phase 1/2 Study of MK-1775 (AZD1775, IND# 121422) in Combination with Oral Irinotecan in Children, Adolescents, and Young Adults with Relapsed or Refractory Solid Tumors (ADVL1312)	16-447/I-II	Steven DuBois, MD, MS 617-632-4450
Relapsed Refractory CNS and Solid Tumors	An Open-Label, Dose-Escalation, Phase I/II Study to Investigate the Safety, PK, PD and Clinical Activity of the MEK Inhibitor Trametinib in Children and Adolescents with Cancer or Plexiform Neurofibromas and Trametinib in Combination with Dabrafenib in Children and Adolescents with Cancers Harboring V600 mutations	15-079/I-II	Mark Kieran, MD, PhD 617-632-4386
HEMATOLOGIC MALIGNANCIES			
Leukemia (ALL) (Relapsed/ Refractory)	A Feasibility Trial of Everolimus (RAD001), an mTor Inhibitor, Given in Combination with Multiagent Re-Induction Chemo-Therapy in Pediatric Patients with Relapsed Acute Lymphoblastic Leukemia (ALL)	11-237/I	Lewis Silverman, MD 617-632-6191
Leukemia (ALL and AML) (Relapsed/Refractory)	Phase I Trial of the Selective Inhibitor of Nuclear Export, KPT-330, in Relapsed Childhood ALL and AML	13-563/I	Andrew Place, MD, PhD 617-632-2313
Hematologic Malignancy: Relapsed ALL and NHL	A Phase I Trial of Temozolomide in Combination with Etoposide and Cyclophosphamide in Children with Relapsed Acute Lymphoblastic Leukemia and Non-Hodgkins Lymphoma	15-213/I	Lewis Silverman, MD 617-632-6191

Treatment Area	Study Name	ID Number/ Phase	Site Investigator
HEMATOLOGIC MALIGNANCIES (continued)			
Acute Myelogenous Leukemia (AML) (Newly Diagnosed)	AML08: A Phase III Radomized Trial of Clofarabine Plus Cytarabine Versus Conventional Induction Therapy and a Phase II Study of Natural Killer Cell Transplantation in Patients with Newly Diagnosed Acute Myeloid Leukemia (St. Jude)	09-337/III	Barbara Degar, MD 617-632-5186
Leukemia Recurrent/Refractory	Matched Targeted Therapy (MTT) Recommendation for Patients with Recurrent, Refractory, or High Risk Leukemias	15-384	Yana Pikman, MD 617-632-4754
Hemophagocytic Lymphohistiocytosis (HLH) (Reactivated)	A Pilot, Open-Label, Single Arm, Multicenter Study to Explore Safety, Tolerability, Pharmacokinetics and Efficacy of Intravenous Multiple Administrations of NI-0501, an Anti-Interferon Gamma (Anti-IFN γ) Monoclonal Antibody, in Pediatrics Patients with Primary HLH Which Has Reactivated	14-018	Barbara Degar, MD 617-632-5186
Hodgkin Lymphoma (High Risk)	Brentuximab Vedotin Substituting Vincristine in the OEPA/ COPDAC Regimen with Involved Node Radiation Therapy for High Risk Pediatric Hodgkin Lymphoma	14-233/II	Amy Billett, MD 617-632-5640
Langerhans Cell Histiocytosis (LCH)	International Collaborative Treatment Protocol for Children and Adolescents with Langerhans Cell Histiocytosis	13-428	Barbara Degar, MD 617-632-5186
Leukemia (Relapsed/Refractory)	A Phase I Trial of Autologous T-Lymphocytes Genetically Targeted to the B-Cell Specific Antigen CD19 in Pediatric and Young Adult Patients with Relapsed B-Cell Acute Lymphoblastic Leukemia	13-526/I	Steven Margossian, MD, PhD 617-632-4882
Leukemia (AML) (Relapsed/ Refractory)	Dasatinib in Combination with Chemotherapy for Relapsed or Refractory Core Binding Factor Acute Myeloid Leukemia: A Phase I Study	15-581	Andrew Place, MD, PhD 617-632-2313
NON-MALIGNANT HEMATOLOGY			
Aplastic Anemia	Unrelated Donor Transplant Versus Immune Therapy in Pediatric Severe Aplastic Anemia (TransIT) PBMTc NMD1601	P00021716	Akiko Shimamura, MD, PhD 617-919-6109
Beta-thalassemia	A Phase 3, double-blind, randomized, placebo-controlled, multicenter study to determine the efficacy and safety of luspatercept (ACE-536) plus best supportive care versus best supportive care in adults who require regular red-blood cell transfusions due to beta-thalassemia	P00021448/III	Ellis Neufeld, MD, PhD 617-919-2139
Diamond Blackfan Anemia	Pilot Study of the Amino Acid Leucine in the Treatment of Patients with Transfusion-Dependent Diamond Blackfan Anemia	P00009112/I-II	Colin Sieff, MB, Bch 617-919-4241
Hemolytic Anemia	Splenectomy in Congenital Hemolytic Anemia	P00006968	Matthew Heeney, MD 617-355-7700
Pyruvate Kinase Deficiency (PKD)	A Phase 2a, Open Label, Randomized, Dose Regime, Safety, Efficacy, Pharmacokinetics and Pharmacodynamic Study of AG-348 in Adult Patients with Pyruvate Kinase Deficiency (PKD) (AG348-C-003)	P00016857/II	Rachael Grace, MD, MMSc 617-919-2144

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NON-MALIGNANT HEMATOLOGY (continued)			
Sickle Cell Disease	A Phase 3, Multicenter, Randomized, Double-Blind, Placebo-Controlled, Parallel-Group Study to Evaluate the Efficacy and Safety of Rivipansel (GMI-1070) in the Treatment of Vaso-Occlusive Crisis in Hospitalized Subjects with Sickle Cell Disease	P0017292/III	Matthew Heeney, MD 617-355-7700
Sickle Cell Disease	GBT440 In Adolescents with Sickle Cell Disease	P00022598	Venee Tubman, MD 617-632-4771
Thrombosis	A Randomized, Open-Label, Active Controlled, Safety and Extrapolated Efficacy Study of apixaban in Pediatric Patients Requiring Anticoagulation for the Treatment of a Venous Thromboembolic Event (B0661037/CV185-325)	P00016074	Cameron Trenor, MD 617-919-3242
Thrombosis	Phase 1, Open-label, Single-dose, Non-randomized study to Evaluate Pharmacokinetic (PK) and Pharmacodynamics (PD) of Edoxaban in Pediatric Patients DU176B-A-U157	P00013392/I	Cameron Trenor, MD 617-919-3242
VASCULAR ANOMALIES			
Vascular Anomalies	A Randomized Phase 2 Study of Vincristine vs. Sirolimus to treat High Risk Kaposiform Hemangioendothelioma – SIR-DA-1202	P00013131	Denise Adams, MD 617-355-5226
Vascular Anomalies	Lymphatic Anomalies Registry	P00007182/II	Cameron Trenor, MD 617-919-3242
STEM CELL TRANSPLANTATION			
Stem Cell Transplantation (Metabolic Disorders)	A Phase 1 Trial of a Single ProHema®-CB Product (Ex Vivo Modulated Human Cord Blood Cells) as Part of Single Cord Blood Unit Transplant after Busulfan/Cyclophosphamide/ ATG Conditioning for Pediatric Patients with Inherited Metabolic Disorders	15-382/I	Christine Duncan, MD 617-632-4882
Stem Cell Transplantation (Dyskeratosis Congenita)	Non-TBI and Alkylator-Free Conditioning for Allogeneic Bone Marrow Transplantation for Bone Marrow Failure Due to Dyskeratosis Congenita / Telomere Disease	P00003466	Suneet Agrawal, MD 617-919-4610
Stem Cell Transplantation (Hematologic Malignancies)	A Phase 1 Trial of a Single ProHema®-CB Product (Ex Vivo Modulated Human Cord Blood Cells) Following Myeloablative Conditioning for Pediatric Patients with Hematologic Malignancies	14-500/I	Christine Duncan, MD 617-632-4882
Stem Cell Transplantation (Graft vs Host Disease)	Abatacept Combined with a Calcineurin Inhibitor and Methotrexate for GVHD Prophylaxis – A Randomized Control Trial	14-327	Christine Duncan, MD 617-632-4882
Stem Cell Transplantation (Hematologic Malignancies)	Multi-Center, Open-Label Randomized Study of Single or Double Myeloablative Cord Blood Transplantation with or without Infusion of Off-the-Shelf Ex Vivo Expanded Cryopreserved Cord Blood Progenitor Cells in Patients with Hematologic Malignancies	13-042/II	Christine Duncan, MD 617-632-4882
Stem Cell Transplantation (Late Effects)	Natural History and Biology of Long-Term Late Effects Following Hematopoietic Cell Transplant for Childhood Hematologic Malignancies	14-460	Christine Duncan, MD 617-632-4882

Treatment Area	Study Name	ID Number/ Phase	Site Investigator
STEM CELL TRANSPLANTATION (continued)			
Stem Cell Transplantation (Cord Blood Unit Access)	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	11-297	Leslie Lehmann, MD 617-632-4882
Stem Cell Transplantation (Gut decontamination)	A Randomized Phase 2 Study to Examine the Impact of Gut Decontamination on Intestinal Microbiome Composition in Pediatric Allogeneic Hematopoietic Stem Cell Transplant Patients	15-394/II	Jennifer Whangbo, MD, PhD 617-632-2664
CELL AND GENE THERAPY FOR RARE DISEASES			
Acute Lymphoblastic Leukemia (Relapsed)	A Phase I Trial of Autologous T-Lymphocytes Genetically Targeted to the B-Cell Specific Antigen CD19 in Pediatric and Young Adult Patients with Relapsed B-Cell Acute Lymphoblastic Leukemia	13-526/I	Steven Margossian, MD, PhD 617-632-4882
Childhood Cerebral Adrenoleukodystrophy (CCALD)	A Phase 2/3 Study of the Efficacy and Safety of Hematopoietic Stem Cells Transduced with Lenti-D Lentiviral Vector for the Treatment of Childhood Cerebral Adrenoleukodystrophy (CCALD)	P00007954/ II-III	David A. Williams, MD 617-919-2697
Severe Combined Immuno-deficiency Syndrome (SCID)	Gene Transfer for SCID-X1 Using a Self-Activating (SIN) Gammaretroviral Vector	09-05-0254	Sung-Yun Pai, MD 617-919-2508
X-Linked Chronic Granulomatous Disease (CGD)	A Phase I/II, Non Randomized, Multicenter, Open-Label Study of G1XCGD (Lentiviral Vector Transduced CD34+ Cells) in Patients with X-Linked CGD	P00012407/II-II	David A. Williams, MD 617-919-2697
Wiskott-Aldrich Syndrome	Pilot and Feasibility Study of Hematopoietic Stem Cell Gene Transfer for the Wiskott-Aldrich Syndrome	P00000148	Sung-Yun Pai, MD 617-919-2508
Stem Cell Transplantation (Hematologic Malignancies)	Phase I/II study of CaspaCide T Cells from an HLA - partially matched family donor after negative selection of TCR $\alpha\beta$ +Tcell in pediatric patients affected by hematological disorders.	15-094/I-II	Susanne Baumeister, MD 617-632-4687
Hemophilia B	Safety and Dose Finding Study of DTX101 in Adults With Moderate/Severe to Severe Hemophilia B	P00020457/II-II	Stacy Croteau, MD, MMS 617-632-3041
SOLID TUMORS			
Solid Tumors Recurrent/Refractory	Multicenter Cohort Study To Evaluate Outcomes after Receipt of Targeted Therapy Matched to an Individualized Cancer Therapy (iCat) Recommendations in Children and Young Adults with Recurrent, Refractory, or High Risk Solid Tumors: The iCat2, GAIN Consortium Study	15-169	Katherine Janeway, MD 617-632-4994
Solid Tumors (Relapsed/Refractory)	A Phase I, Open-Label, Dose Escalation Study of LDK 378 in Pediatric Patients with Malignancies that Have a Genetic Alteration in Anaplastic Lymphoma Kinase (ALK)	13-180/I	Suzanne Shusterman, MD 617-632-4901
Ewing Sarcoma (Recurrent)	Phase I Study of Olaparib with Irinotecan and/or Temozolomide in Adolescent and Adult Patients with Recurrent/Metastatic Ewing's Sarcoma following failure of prior chemotherapy	13-115/I	Steven DuBois, MD, MS 617- 632-4450

Treatment Area	Study Name	ID Number/ Phase	Site Investigator
SOLID TUMORS (continued)			
Neuroblastoma (Relapsed/Refractory)	Randomized Phase II Study of 131I-MIBG vs. 131I-MIBG with Vincristine and irinotecan vs. 131I-MIBG with Vorinostat for Resistant or Relapsed Neuroblastoma (NANT 2011-01)	14-184/II	Suzanne Shusterman, MD 617-632-4901
Neuroblastoma (Relapsed/Refractory)	Phase I Study of Difluoromethylornithine (DFMO) and Celecoxib with Cyclophosphamide/Topotecan for Patients with Relapsed or Refractory Neuroblastoma (NANT 2012-01)	13-481/I	Suzanne Shusterman, MD 617-632-4901
Solid Tumors (Relapsed/Refractory)	A Phase I, Open-Label, Dose Escalation Study of LDK 378 in Pediatric Patients with Malignancies that Have a Genetic Alteration in Anaplastic Lymphoma Kinase (ALK)	13-180/I	Suzanne Shusterman, MD 617-632-4901
Neuroblastoma, (Relapsed/Refractory)	A Phase I Study of Lenalidomide and Anti-GD2 Mab Ch14.18 +/- Isotretinoin in Patients with Refractory or Recurrent Neuroblastoma. (NANT N2011-04)	12-505/I	Suzanne Shusterman, MD 617-632-4901
Neuroblastoma Relapsed/Refractory	NANT 2014-01: PHASE I STUDY OF SF1126 FOR PATIENTS WITH RELAPSED OR REFRACTORY NEUROBLASTOMA	15-243/I	Suzanne Shusterman, MD 617-632-4901
Neuroblastoma Relapsed/Refractory	NANT N2013-02 A Phase I Study of Sorafenib and Cyclophosphamide/Topotecan in Patients with Relapsed and Refractory Neuroblastoma	14-494/I	Suzanne Shusterman, MD 617-632-4901
Malignant Germ Cell Tumors	A Phase II Study of Sirolimus & Erlotinib in Recurrent/Refractory Malignant Germ Cell Tumors	14-134	Lindsay Frazier, MD 617-632-2237
BRAIN TUMORS			
Medulloblastoma (Newly Diagnosed Wnt Positive)	Pilot Study of Surgery and Chemotherapy-Only Approach in the Upfront Therapy of Children with Wnt Positive Standard Risk Medulloblastoma	14-252/Pilot	Pratiti Bandopadhyay, MBBS, PhD 617-632-4309
Medulloblastoma (Recurrent/Refractory)	A Phase II Study of Metronomic and Targeted Anti-Angiogenesis Therapy for Children with Refractory/Recurrent Medulloblastoma	13-359/II	Mark W. Kieran, MD, PhD 617-632-4386
Low Grade Glioma (Previously Untreated)	A Phase III Study Comparing Two Carboplatin Containing Regimens for Children and Young Adults with Previously Untreated Low Grade Glioma	15-117/III	Peter Manley, MD 617-632-6975
Brain Tumors (Relapsed/Refractory)	PNOC 002: Safety, Phase 0, and Pilot Efficacy Study of Vemurafenib, an oral inhibitor of BRAFV600E, in Children and Young Adults with Recurrent/Refractory BRAFV600E or BRAF Ins T mutant Brain Tumors	16-234/Pilot	Susan Chi, MD 617-632-4386
Neurofibromatosis Type 1	A Phase II Study of Cabozantinib (XL184) for Plexiform Neurofibromas in Subjects with Neurofibromatosis Type 1 Age 16 Years or Greater	14-219/II	Nicole Ullrich, MD, PhD 617-355-3193
Neurofibromatosis Type 1, Plexiform Neurofibromas	A Phase II Trial of Peginterferon alfa-2b (Pegintron) for Neurofibromatosis Type 1 Related Unresectable, Symptomatic or Life-Threatening Plexiform Neurofibromas	08-330/II	Mark W. Kieran, MD, PhD 617-632-4368

Dana-Farber/Boston Children's Cancer and Blood Disorders Center represents the integrated pediatric oncology program at Dana-Farber Cancer Institute and Boston Children's Hospital. The more than 60-year-old program offers comprehensive care to children, adolescents, young adults, and survivors of all types of childhood cancers and related blood diseases.