

Selected Protocols for the Treatment of Pediatric Hematologic and Oncologic Disorders

Treatment Group	ID Number	Phase	Study Name	Site Investigator
Brain Tumor	05-303	Pilot	A Pilot Non-Therapeutic Neuro-Imaging Study of 18F-FLT in Pediatric Patients with Newly Diagnosed Central Nervous System Tumors	Mark W. Kieran, MD, PhD 617-632-4368
Brain Tumor	06-092	I	A Phase I Clinical Trial of AZD2171 in Children with Recurrent or Progressive Central Nervous System (CNS) Tumors (PBTC-020)	Mark W. Kieran, MD, PhD 617-632-4368
Brain Tumor	07-125	III	A Phase III Study Investigating the Efficacy of Carboplatin Administered Concomitantly with Radiation and Isotretinoin as a Pro-Apoptotic Agent in Other Than Average Risk Medulloblastoma/PNET Patients (COG ACNS0332)	Susan Chi, MD 617-632-4368
Brain Tumor/ Stem Cell Transplantation	07-148	II	High Dose Temozolomide, Thiotepa and Carboplatin with Autologous Stem Cell Rescue Followed by Continuation Therapy with 13-cis-retinoic Acid in Patients with Recurrent/Refractory Malignant Brain Tumors	Leslie Lehmann, MD 617-632-4932
Brain Tumor	07-308	III	Randomized Phase III Study for Patients with High Risk PNET/Medulloblastoma under the Age of 36 Months (COG ACNS0334)	Susan Chi, MD 617-632-4368
Brain Tumor	08-339	I	A Multi-Center, Open-Label, 5-Part Study to Evaluate the Pharmacokinetics, Safety, and Tolerability of Aprepitant and Fosaprepitant Dimeglumine in Pediatric Patients Receiving Emetogenic Chemotherapy	Mark W. Kieran, MD, PhD 617-632-4368
Brain Tumor	09-001	II	A Phase II Study of RAD001 (Everolimus) for Children with Chemotherapy-Refractory Progressive or Recurrent Low-Grade Gliomas (POETIC)	Mark W. Kieran, MD, PhD 617-632-4907
Fanconi Anemia/Stem Cell Transplantation	08-377	II	A Multicenter Phase II Trial of Hematopoietic Stem Cell Transplantation for the Treatment of Patients with Fanconi Anemia Lacking a Genotypically Identical Donor, Using a Chemotherapy Only Cytoreduction with Busulfan, Cyclophosphamide and Fludarabine	David A. Williams, MD 617-919-2697

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Fanconi Anemia/ Dyskeratosis Congenita	09-03-0131	I/II	Phase I/II Dose Escalation Trial of Danazol in Patients with Fanconi Anemia or Dyskeratosis Congenita	Colin Sieff, MB BCh 617-919-4241
Hemophilia	08-02-0054	II	Randomized, Controlled Trial of Kogenate FS Eonjugated to PEG-Liposomes Compared to Kogenate FS Alone for Prophylactic Therapy in Severe Factor VIII Deficiency	Ellis Neufeld, MD 617-919-2139
Hemophilia	X08-05-0209	IV	Dosing Observational Study in Hemophilia Subjects with Inhibitors: A Phase IV Diary Study in Subjects Prescribed NovoSeven as First Line on Demand Therapy for Acute Bleeding	Ellis Neufeld, MD 617-919-2139
Hodgkins Lymphoma	01-273	III	Risk-Adapted Therapy in Pediatric Hodgkins Disease	Amy Billett, MD 617-632-5640
Hodgkins Lymphoma	06-309	III	Stanford V Chemotherapy with Low-Dose Tailored-Field Radiation Therapy for Intermediate Risk Pediatric Hodgkins Disease (HOD05)	Amy Billett, MD 617-632-5640
Idiopathic Thrombocytopenic Purpura	03-08-058		North American Chronic ITP Registry	Ellis Neufeld, MD 617-919-2139
Idiopathic Thrombocytopenic Purpura	06-03-0134		Genetic Modifiers of Childhood Chronic Immune Thrombocytopenic Purpura	Ellis Neufeld, MD 617-919-2139
Leukemia (ALL)	05-001	III	Treatment of Acute Lymphoblastic Leukemia in Children	Lewis Silverman, MD 617-632-5285
Leukemia (ALL)	05-232	Pilot/II	A Feasibility Pilot and Phase II Study of Chemoimmunotherapy with Epratuzumab for Children with Relapsed CD22-Positive Acute Lymphoblastic Leukemia (COG ADVL04P2)	Lewis Silverman, MD 617-632-5285
Leukemia (ALL)	05-388	II	A Phase II Dose-Escalation Study of Clolarä (Clofarabine) Plus Etoposide and Cyclophosphamide in Pediatric Patients with Refractory or Relapsed Acute Leukemias	Lewis Silverman, MD 617-632-5285
Leukemia (Refractory)	06-166	I	A Phase I Study of the RAF Kinase and Receptor Tyrosine Kinase Inhibitor Sorafenib (BAY 43-9006, NSC# 724772 IND# 69896) in Children with Refractory Solid Tumors or Refractory Leukemias (COG ADVL0413)	Suzanne Shusterman, MD 617-632-4901
Leukemia (Infant ALL)	06-172	III	Interfant-06 – International Collaborative Treatment Protocol for Infants under One Year with Acute Lymphoblastic Leukemia or Biphenotypic Leukemia	Lewis Silverman, MD 617-632-5285
Leukemia (ALL)	06-429	n/a	Rapamycin in Adults and Children with Relapsed ALL	Krysta Schlis, MD 617-632-2678

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Leukemia (ALL)	09-019	II	A Study of Bortezomib with Chemotherapy for Relapsed/Refractory Acute Lymphoblastic Leukemia	Lewis Silverman, MD 617-632-5285
Leukemia (ALL/AML)	09-238	I	A Phase I, Open-Label, Dose-Escalating Study to Evaluate the Safety, Tolerability and Pharmacokinetics of Twice Daily Oral Midostaurin and to Evaluate the Preliminary Clinical and Pharmacodynamic Response in Pediatric Patients with Relapsed or Refractory Leukemia	Lewis Silverman, MD 617-632-6191
Leukemia (AML)	09-278	II	A Phase II Pilot Study of Bortezomib (PS-341, Velcade, IND# 58,443) Combined with Reinduction Chemotherapy in Children and Young Adults with Recurrent, Refractory or Secondary Acute Myeloid Leukemia	Barbara Degar, MD 617-632-6801
Leukemia (AML)	09-313	III	The Treatment of Down Syndrome Children with Acute Myeloid Leukemia (AML) and Myelodysplastic Syndrome (MDS) Under the Age of 4 Years. A Group Wide Phase III Study	Barbara Degar, MD 617-632-6801
Hemophagocytic Lymphohistiocytosis (HLH)	05-137	Pilot	HLH- 2004 – Treatment Protocol of the 2nd International HLH Study	Barbara Degar, MD 617-632-6801
Progeria	07-01-0007	II	An Open Label Dose Adjusted Phase II Trial of the Oral Farnesyltransferase Inhibitor (FTI) Lonafarnib (SCH66336) for Patients with Hutchinson-Gilford Progeria Syndrome (HGPS) and Progeroid Laminopathies	Mark W. Kieran, MD, PhD 617-632-4368
Neuroblastoma	04-112	III	Phase III Randomized Study of Chimeric Antibody 14.18 (Ch14.18) in High Risk Neuroblastoma Following Myeloblastic Therapy and Autologous Stem Cell Rescue	Suzanne Shusterman, MD 617-632-4901
Neuroblastoma	05-147	II	I-Metaiodobenzylguanidine (131 I-MIBG) with Intensive Chemotherapy and Autologous Stem Cell Rescue for High-Risk Neuroblastoma (NANT01-02)	Suzanne Shusterman, MD 617-632-4901
Neuroblastoma	05-302	I	Phase I Study of Fenretinide (4-HPR, NSC 374551) Lym-X-sorb™(LXS) Oral Powder in Patients with Recurrent or Resistant Neuroblastoma (NANT 04-04)	Suzanne Shusterman, MD 617-632-4901
Neuroblastoma	06-272	I	Irinotecan and Vincristine for Radiosensitization of (131)I-MIBG Therapy in Resistant/Relapsed High-Risk Neuroblastoma (NANT04-06)	Suzanne Shusterman, MD 617-632-4901
Neuroblastoma	07-046	I	A Phase I Study of Intravenous (Emulsion) Fenretinide (4-HPR, NSC 374551) in Children with Recurrent or Resistant Neuroblastoma (NANT04-03)	Suzanne Shusterman, MD 617-632-4901

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Neuroblastoma	07-345	III	Response- and Biology-Based Therapy for Intermediate-risk Neuroblastoma (COG)	Suzanne Shusterman, MD 617-632-490
Neuroblastoma	08-111	Other	An Open Label, Expanded Access Protocol Using 131I-Metaiodobenzylguanidine (131I-MIBG) Therapy in Patients with Refractory Neuroblastoma	Suzanne Shusterman, MD 617-632-4901
Neuroblastoma	09-078	I	A Phase I Study of Bevacizumab with Bolus and Metronomic Cyclophosphamide and Zoledronic Acid in Children with Recurrent or Refractory Neuroblastoma (NANT 07-02)	Suzanne Shusterman, MD 617-632-4901
Neuroblastoma	09-225	I	Modulation of Intensive Melphalan (L-PAM) by Buthionine Sulfoximine (BSO) with Autologous Stem Cell Support for Resistant/Recurrent High-Risk Neuroblastoma (NANT99-02)	Suzanne Shusterman, MD 617-632-4901
Neurofibromatosis	08-035	II	Phase II Study of mTOR Inhibitor Sirolimus for Plexiform Neurofibromas	Nicole Ullrich, MD, PhD 617-355-2066
Neurofibromatosis	08-330	II	A Phase II Trial of Peginterferon alfa-2b (Pegintron) for Neurofibromatosis Type 1 Related Unresectable, Symptomatic or Life-Threatening Plexiform Neurofibromas	Mark W. Kieran, MD, PhD 617-632-4368
Neurofibromatosis	09-05-0228		A Randomized Placebo-Controlled Study of Lovastatin in Children with Neurofibromatosis Type I (for Children with Cognitive and Attention Problems)	Nicole Ullrich, MD, PhD 617-355-2066
Sickle Cell Disease	06-11-0498	II	Stroke with Transfusions Switching to Hydroxyurea (SWITCH): A Phase II Randomized Clinical Trial to Compare Standard Therapy (Erythrocyte Transfusions with Iron Chelation) with Alternative Therapy (Hydroxyurea with Phlebotomy) for the Prevention of Secondary Stroke and the Management of Iron Overload in Pediatric Subjects with Sickle Cell Anemia and Previous Stroke	Matthew Heeney, MD 617-355-7700
Sickle Cell Disease	09-05-0252	II	Preventing Acute Chest Syndrome by Transfusion (PROACTIVE) Feasibility Study	Matthew Heeney, MD 617-355-7700
Solid Tumor	06-286	I	A Phase I Study of Sunitinib (SU11248), an Oral Multi-Targeted Tyrosine Kinase Inhibitor, in Children with Refractory Solid Tumors (COG ADVL0612)	Suzanne Shusterman, MD 617-632-4901
Solid Tumor	08-179	I	A Phase I Study of VEGF Trap (NSC# 724770, IND# 100137) in Children with Refractory Solid Tumors	Suzanne Shusterman, MD 617-632-4901

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Solid Tumor/ Refractory Leukemia/ Neuroblastoma	08-309	I/II	A Phase I Study of MLN8237, an Oral Selective Small Molecule Inhibitor of Aurora A Kinase, in Children with Relapsed/Refractory Solid Tumors and Leukemia (COG ADVL0812)	Suzanne Shusterman, MD 617-632-4901
Solid Tumor	09-031	II	A Phase II Study of IMC-A12 (Anti-IGF-I Receptor Monoclonal Antibody, IND# 100947, NSC# 742460) in Children With Relapsed/Refractory Solid Tumors (COG ADVL0821)	Suzanne Shusterman, MD 617-632-4901
Solid Tumor	09-124	I	A Phase I Study of IMC-A12 (Anti-Insulin-Like Growth Factor-I Receptor Monoclonal Antibody) in Combination with CCI-779 (Temsirolimus) in Pediatric Patients with Recurrent or Refractory Solid Tumors (COG ADVL0813)	Suzanne Shusterman, MD 617-632-4901
Solid Tumor	09-251	I	A Phase I Study of Pazopanib as a Single Agent for Children with Refractory Solid Tumors (COG ADVL0815)	Suzanne Shusterman, MD 617-632-4901
Solid Tumor/Leukemia	09-252	I	A Phase I Study of Obatoclox (Pan Anti-Apoptotic BCL-2 Family Small Molecule Inhibitor), in Combination with Vincristine/Doxorubicin/Dexrazoxane, in Children with Relapsed/Refractory Solid Tumors or Leukemia (COG ADVL0816)	Suzanne Shusterman, MD 617-632-4901
Solid Tumor/Leukemia	09-347	I/II	A Phase I/II Study of PF-02341066, an Oral Small Molecule Inhibitor of Anaplastic Lymphoma Kinase (ALK) and C-Met, in Children with Relapsed/Refractory Solid Tumors and Anaplastic Large Cell Lymphoma (COG ADVL0912)	Suzanne Shusterman, MD 617-632-4901
Thalassemia or other transfusion dependent anemias	09-08-0411	Ib	A Phase Ib Open Label, Multi-Center, Escalating Multiple Dose Study to Assess the Safety, Tolerability, Pharmacokinetics and Pharmacodynamics of FBS0701 in Patients with Transfusional Iron Overload	Deborah Chirnomas, MD 617-632-6798
Thalassemia	X07-10-0437	IV	A 5-Year Observational Study (Registry) of Children Aged 2 to <6 Years at Enrollment with Transfusional Hemosiderosis Treated with Deferasirox	Ellis Neufeld, MD 617-919-2139
Thalassemia	09-02-0069	IV	A Single-Arm, Open-Label Study of the Palatability and Tolerability of Exjade Taken with Meals, with Different Liquids or Crushed and Added to Food	Deborah Chirnomas, MD 617-632-6798
Thalassemia	03-10-163		Thalassemia Data and Blood Specimen Collection System	Ellis Neufeld, MD 617-919-2139
Thalassemia	06-10-0466		Observational. Accrual complete except for patients undergoing transplant (Thalassemia Longitudinal Cohort)	Ellis Neufeld, MD 617-919-2139

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Stem Cell Transplantation	06-158	I/II	Fludarabine-Based Conditioning for Allogeneic Marrow Transplantation From HLA-compatible Unrelated Donors in Severe Aplastic Anemia (CTN 0301)	Leslie Lehmann, MD 617-632-4932
Stem Cell Transplantation	07-004		Impact of Iron Overload in Patients Undergoing Stem Cell Transplantation	Deborah Chirnomas, MD 617-632-6798
Stem Cell Transplantation	07-031	III	Multi-Center, Open Label, Randomized Trial Comparing Single Versus Double Umbilical Cord Blood (UCB) Transplantation in Pediatric Patients with Leukemia and Myelodysplasia	Leslie Lehmann, MD 617-632-4932
Stem Cell Transplantation	07-031	III	Multi-Center, Open Label, Randomized Trial Comparing Single Versus Double Umbilical Cord Blood (UCB) Transplantation in Pediatric Patients with Leukemia and Myelodysplasia	Leslie Lehmann, MD 617-632-4932
Stem Cell Transplantation	07-238	III	A Phase III Study of Gemtuzumab Ozogamicin (Mylotarg®) Combined with Conventional Chemotherapy for De Novo Acute Myeloid Leukemia (AML) in Children, Adolescents and Young Adults	Leslie Lehmann, MD 617-632-4932
Stem Cell Transplantation	07-368	III	A Randomized Trial of Sirolimus-Base Graft versus Host Disease Prophylaxis after Hematopoietic Stem Cell Transplantation in Selected Patients with CR1 and CR2 ALL	Leslie Lehmann, MD 617-632-4932
Stem Cell Transplantation/ Sickle Cell Disease	09-141	II	Unrelated Donor Hematopoietic Cell Transplantation for Children with Severe Sickle Cell Disease Using a Reduced Intensity Conditioning Regimen	Leslie Lehmann, MD 617-632-4932

Dana-Farber/Children's Hospital Cancer Care investigators also offer access to protocols managed by the following research groups: Children's Oncology Group (Phase I Center), Diamond-Blackfan Anemia Centers, Histiocyte Society, New Approaches to Neuroblastoma Therapy Consortium, New England Pediatric Sickle Cell Consortium, North American Chronic ITP Registry, Pediatric Blood and Marrow Transplant Consortium, Pediatric Oncology Experimental Therapeutic Investigator Consortium, Sickle Cell Disease Clinical Research Network, Thalassemia Clinical Research Network, and the Transfusion Medicine Hemostasis Clinical Research Network.

If you would like additional information about any of our trials, contact the investigator or call 1-888-PEDI-ONC.

Dana-Farber/Children's Hospital Cancer Care represents the integrated pediatric oncology program at Dana-Farber Cancer Institute and Children's Hospital Boston. 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 diseases.