

Selected Protocols for the Treatment of Pediatric Hematologic and Oncologic Disorders

Treatment Area	Study Name	ID Number/ Phase	Site Investigator
BRAIN TUMORS			
Brain Tumor Imaging	A Pilot Non-Therapeutic Neuro-Imaging Study of 18F-FLT in Pediatric Patients with Newly Diagnosed Central Nervous System Tumors	05-303/ Pilot	Mark W. Kieran, MD, PhD 617-632-4368
Recurrent Brain Tumors	A Phase I Clinical Trial of AZD2171 in Children with Recurrent or Progressive Central Nervous System (CNS) Tumors (PBTC-020)	06-092/I	Mark W. Kieran, MD, PhD 617-632-4368
High Grade Glioma	A Phase I Study of AdV-tk + Prodrug Therapy in Combination with Radiation Therapy for newly diagnosed malignant glioma	07-098/I	Mark W. Kieran, MD, PhD 617-632-4368
Medulloblastoma	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)	07-125/III	Susan Chi, MD 617-632-4368
Brain Tumor/Stem Cell Transplantation	High Dose Temozolomide, Thiotepe and Carboplatin with Autologous Stem Cell Rescue Followed by Continuation Therapy with 13-Cis-Retinoic Acid in Patients with Recurrent/Refractory Malignant Brain Tumors	07-148/II	Leslie Lehmann, MD 617-632-4882
Medulloblastoma (infants/toddlers)	Randomized Phase III Study for Patients with High Risk PNET/Medulloblastoma under the Age of 36 Months (COG ACNS0334)	07-308/III	Susan Chi, MD 617-632-4368
Neurofibromatosis/Low Grade Glioma	A Phase II Study of RAD001 (Everolimus) for Children with Neurofibromatosis type I and Chemotherapy-Refractory Progressive or Recurrent Low-Grade Gliomas (DOD)	09-452/II	Mark W. Kieran, MD, PhD 617-632-4907
High Grade Glioma and Diffuse Intrinsic Pontine Glioma	Cetuximab, Irinotecan and XRT for Newly Diagnosed HGG and Diffuse Intrinsic Pontine Glioma	09-123/II	Mark W. Kieran, MD, PhD 617-632-4907
Brain Tumor Supportive care	Xerocept for Control of Tumor or Radiation Mediated Swelling	10-103/I-II	Mark W. Kieran, MD, PhD 617-632-4907
Recurrent ependymoma	Erlotinib vs Etoposide for Recurrent Ependymoma	10-093/II	Mark W. Kieran, MD, PhD 617-632-4907
Recurrent Low Grade Glioma	A Phase II Study of RAD001 (Everolimus) for Children with Chemotherapy-Refractory Progressive or Recurrent Low-Grade Gliomas (POETIC)	09-001/II	Mark W. Kieran, MD, PhD 617-632-4907
Tuberous Sclerosis and astrocytoma	A Randomized, Double-Blind, Placebo-Controlled Study of RAD001 in the Treatment of Patients with Subependymal Giant Cell Astrocytomas (SEGA) Associated with Tuberous Sclerosis Complex (TSC)	09-295/III	Mustafa Sahin, MD, PhD 617-919-4518
BLOOD DISEASES			
Fanconi Anemia (Stem Cell Transplantation)	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	08-377/II	David A. Williams, MD 617-919-2697
Fanconi Anemia/ Dyskeratosis Congenia	Phase I/II Dose Escalation Trial of Danazol in Patients with Fanconi Anemia or Dyskeratosis Congenia	09-03- 0131/I-II	Colin Sieff, MB BCH 617-919-4241
Hemophilia	Randomized, Controlled Trial of Kogenate FS Eonjugated to PEG-Liposomes Compared to Kogenate FS Alone for Prophylactic Therapy in Severe Factor VIII Deficiency	08-02- 0054/II	Ellis Neufeld, MD, PhD 617-919-2139
Hemophilia	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	X08-05- 0209/IV	Ellis Neufeld, MD, PhD 617-919-2139

BLOOD DISEASES			
Hemophilia	NN7128-1907 (An Exploratory Multi-Centre, Multi-National, Randomized, Double Blinded, Parallel Arm Trial Evaluating Safety, Pharmacokinetics and Dose-finding of prophylactic administration of Long Acting rFVIIa (LA-rFVIIa) in Haemophilia A or B Patients With Inhibitors).	09-08-0414	Kapil Saxena, MD 617-355-8246
Hemophilia	Health-Related Quality of Life in Patients Requiring Chronic Red Cell Transfusions: Validation Study (TranQOL)	X10-05-0249	Ellis Neufeld, MD, PhD 617-919-2139
Hemophilia	Rituximab for the Treatment of Patients with Congenital Hemophilia A and High Titer Inhibitors	05-09-136	Ellis Neufeld, MD, PhD 617-919-2139
Idiopathic Thrombocytopenic Purpura	North American Chronic ITP Registry	X03-08-058	Ellis Neufeld, MD, PhD 617-919-2139
Idiopathic Thrombocytopenic Purpura	Genetic Modifiers of Childhood Chronic Immune Thrombocytopenic Purpura	06-03-0134	Ellis Neufeld, MD, PhD 617-919-2139
Idiopathic Thrombocytopenic Purpura	A Three-Part, Staggered Cohort, Open-Label and Double-Blind, Randomized, Placebo Controlled Study to Investigate the Efficacy, Safety, Tolerability and Pharmacokinetics of Eltrombopag, a Thrombopoietin Receptor Agonist, in Previously Treated Pediatric Patients with Chronic Idiopathic Thrombocytopenic Purpura (ITP)	10-02-0056/III	Ellis Neufeld, MD, PhD 617-919-2139
Myelodysplastic Syndrome/Bone Marrow Failure Disorders	Pediatric Myelodysplastic Syndrome and Bone Marrow Failure Disorder Patient Registry and Tissue Repository	10-02-0057	Inga Hofmann, MD 617-355-2457
Iron overload	A Phase 2, 24 week, randomized, open label, multi-center study to assess the safety, tolerability, and pharmacodynamics of FBS0701 in the treatment of chronic iron overload requiring chelation therapy	10-07-0329/II	Rachael Grace, MD 617-632-4542
Sickle Cell Disease, stroke prevention	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	06-11-0498/II	Matthew Heeney, MD 617-355-7700
Sickle Cell Disease	Preventing Acute Chest Syndrome by Transfusion (PROACTIVE) Feasibility Study	09-05-0252/II	Matthew Heeney, MD 617-355-7700
Sickle Cell Disease	Safety of Adenosine 2A Agonist Lexiscan in Children and Adults with Sickle Cell Disease	09-308/I	David G. Nathan, MD 617-632-2155 Matthew Heeney, MD 617-355-7700
Sickle Cell Disease	Improving Pain Management Outcomes with Various Strategies of Patient-Controlled Analgesia	10-01-0024/III	Matthew Heeney, MD 617-355-7700
Thalassemia	A 5-Year Observational Study (Registry) of Children Aged 2 to <6 Years at Enrollment with Transfusional Hemosiderosis Treated with Deferasirox	X07-10-0437/IV	Ellis Neufeld, MD, PhD 617-919-2139
Thalassemia	Thalassemia Data and Blood Specimen Collection System	03-10-163	Ellis Neufeld, MD, PhD 617-919-2139
Thalassemia	Thalassemia Longitudinal Cohort (Accrual Complete Except for Patients Undergoing Transplant)	06-10-0466/ Observational	Ellis Neufeld, MD, PhD 617-919-2139
Thalassemia	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	09-02-0069/IV	Ellis Neufeld, MD, PhD 617-919-2139
Thalassemia or other Transfusion-Dependent Anemias	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	09-08-0411/Ib	Ellis Neufeld, MD, PhD 617-919-2139
LEUKEMIA / LYMPHOMA			
Hodgkins Lymphoma (low risk)	Risk-Adapted Therapy in Pediatric Hodgkins Disease	01-273/III	Amy Billett, MD 617-632-5640
Hodgkins Lymphoma (intermediate risk)	Stanford V Chemotherapy with Low-Dose Tailored-Field Radiation Therapy for Intermediate Risk Pediatric Hodgkins Disease (HOD05)	06-309/III	Amy Billett, MD 617-632-5640

LEUKEMIA / LYMPHOMA			
Hodgkins Lymphoma (high risk)	Reduced Duration Stanford v Chemotherapy with Low-Dose Tailored-Field Radiation Therapy for Favorable Risk Pediatric Hodgkin Lymphoma (HOD08)	09-273/II	Amy Billett, MD 617-632-5640
Leukemia (ALL) newly diagnosed	Treatment of Acute Lymphoblastic Leukemia in Children	05-001/III	Lewis Silverman, MD 617-632-5285
Leukemia (Infant ALL), newly diagnosed	Interfant-06 – International Collaborative Treatment Protocol for Infants under One Year with Acute Lymphoblastic Leukemia or Biphenotypic Leukemia	06-172/III	Lewis Silverman, MD 617-632-5285
Leukemia (ALL), relapsed/refractory	A Phase I Trial of Nelarabine, Cyclophosphamide, Etoposide in Relapsed, Refractory T-Cell ALL	10-052/I	Lewis Silverman, MD 617-632-5285
Leukemia (ALL), relapsed/refractory	A Phase I Trial of CAT-8015 (Toxin-conjugated anti-CD22) for Relapsed, Refractory B-precursor ALL	09-340/I	Lewis Silverman, MD 617-632-5285
Leukemia (ALL/AML), relapsed/refractory	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	09-238/I	Lewis Silverman, MD 617-632-5285
Leukemia (ALL), relapsed/refractory	A Study of Bortezomib with Chemotherapy for Relapsed/Refractory Acute Lymphoblastic Leukemia	09-019/II	Lewis Silverman, MD 617-632-5285
Leukemia (ALL), relapsed/refractory	An Open-Label, Multi-Center Safety and Tolerability Pilot Combination Study of Clofarabine, Etoposide, Cyclophosphamide, PEG-Asparaginase, and Vincristine in Pediatric Patients with Acute Lymphoblastic Leukemia in First Relapse	09-371/I	Krysta Schlis, MD 617-632-2678
Leukemia (ALL), relapsed/refractory	A Feasibility Pilot and Phase II Study of Chemoimmunotherapy with Epratuzumab for Children with Relapsed CD22-Positive Acute Lymphoblastic Leukemia (COG ADVL04P2)	05-232/ Pilot/II	Lewis Silverman, MD 617-632-5285
Leukemia (refractory)	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)	06-166/I	Suzanne Shusterman, MD 617-632-4901
Leukemia (ALL), relapsed/refractory	Rapamycin in Adults and Children with Relapsed Acute Lymphoblastic Leukemia	06-429	Krysta Schlis, MD 617-632-2678
Leukemia (AML), relapsed/refractory	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	09-313/III	Barbara Degar, MD 617-632-6801
Leukemia (AML)	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	09-278/II	Barbara Degar, MD 617-632-6801
Leukemia (AML)	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	09-313/III	Barbara Degar, MD 617-632-6801
Leukemia (AML)	AML08: A Phase III Randomized 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 617-632-6801
NEUROBLASTOMA			
Neuroblastoma, high risk	Safety Trial of Chimeric anti-GD2 Antibody	10-077/III	Suzanne Shusterman, MD 617-632-4901
Neuroblastoma, high risk	Phase III Randomized Trial of Single vs. Tandem Myeloablative as Consolidation Therapy for High-Risk Neuroblastoma	07/304/III	Suzanne Shusterman, MD 617-632-4901
Neuroblastoma, OMS	Use of Intravenous Gammaglobulin Therapy for Patients with Neuroblastoma Associated Opsoclonus-Myoclonus-Ataxia Syndrome Treated with Chemotherapy and Prednisone	06-184/III	Suzanne Shusterman, MD 617-632-4901
Neuroblastoma, relapsed	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)	05-302/I	Suzanne Shusterman, MD 617-632-4901
Neuroblastoma, intermediate risk	Response- and Biology-Based Therapy for Intermediate-Risk Neuroblastoma (COG)	07-345/III	Suzanne Shusterman, MD 617-632-4901
Neuroblastoma, relapsed	A Phase I Study of Intravenous (Emulsion) Fenretinide (4-HPR, NSC 374551) in Children with Recurrent or Resistant Neuroblastoma (NANT04-03)	07-046/I	Suzanne Shusterman, MD 617-632-4901

NEUROBLASTOMA			
Neuroblastoma, relapsed/refractory	An Open Label, Expanded Access Protocol Using 131I-Metaiodobenzylguanidine (131 I-MIBG) Therapy in Patients with Refractory Neuroblastoma	08-111	Suzanne Shusterman, MD 617-632-4901
Neuroblastoma, relapsed	Vorinostat +131 I-MIBG for Relapsed Neuroblastoma	10-051/I	Suzanne Shusterman, MD 617-632-4901
Neuroblastoma, relapsed	A Phase I Study of Bevacizumab with Bolus and Metronomic Cyclophosphamide and Zoledronic Acid in Children with Recurrent or Refractory Neuroblastoma (NANT 07-02)	09-078/I	Suzanne Shusterman, MD 617-632-4901
Neuroblastoma, relapsed	Modulation of Intensive Melphalan (L-PAM) by Buthionine Sulfoximine (BSO) with Autologous Stem Cell Support for Resistant/Recurrent High-Risk Neuroblastoma (NANT99-02)	09-225/I	Suzanne Shusterman, MD 617-632-4901
SOLID TUMOR			
Solid Tumor, relapsed	A Phase I Study of Sunitinib (SU11248), an Oral Multi-Targeted Tyrosine Kinase Inhibitor, in Children with Refractory Solid Tumors (COG ADVL0612)	06-286/I	Suzanne Shusterman, MD 617-632-4901
Solid Tumor, relapsed	A Phase I Study of VEGF Trap (NSC# 724770, IND# 100137) in Children with Refractory Solid Tumors	08-179/I	Suzanne Shusterman, MD 617-632-4901
Solid Tumor/ Refractory Leukemia/ Neuroblastoma	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)	08-309/I-II	Suzanne Shusterman, MD 617-632-4901
Solid Tumor, relapsed	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)	09-031/I	Suzanne Shusterman, MD 617-632-4901
Solid Tumor, relapsed	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)	09-124/I	Suzanne Shusterman, MD 617-632-4901
Solid Tumor, relapsed	A Phase I Study of Pazopanib as a Single Agent for Children with Refractory Solid Tumors (COG ADVL0815)	09-251/I	Suzanne Shusterman, MD 617-632-4901
Solid Tumor/Leukemia, relapsed	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)	09-252/I	Suzanne Shusterman, MD 617-632-4901
Solid Tumor/Lymphoma, relapsed	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)	09-347/I-II	Suzanne Shusterman, MD 617-632-4901
Solid Tumor, relapsed	A Phase I Study of Vorinostat and Bortezomib in Children with Refractory or Recurrent Solid Tumors, Including CNS Tumors and Lymphomas (COG ADVL0916)	09-375/I	Suzanne Shusterman, MD 617-632-4901
Solid Tumor, relapsed	A Phase 1/2 Study of EZN-2208 in Children, Adolescents, and Young Adults with Relapsed or Refractory Solid Tumors	10-010/I-II	Suzanne Shusterman, MD 617-632-4901
Solid Tumor, relapsed	A Phase 1 Study of RO4929097, an Oral Small Molecule Inhibitor of Gamma-Secretase, in Children with Relapsed/Refractory Solid or CNS Tumors, Lymphoma, or T-cell Leukemia	10-229 /I	Suzanne Shusterman, MD 617-632-4901
Solid Tumor, relapsed	A Phase I Study of Irinotecan, Temozolomide and Temsirolimus in Children with Refractory or Recurrent Solid Tumors	10-248/I	Suzanne Shusterman, MD 617-632-4901
Solid Tumor, relapsed	A Phase I Dose Escalation Study of Seneca Valley Virus (NTX-010), a Replication-Competent Picornavirus, in Relapsed/Refractory Pediatric Patients with Neuroblastoma, Rhabdomyosarcoma, or Rare Tumors with Neuroendocrine Features	10-243/I	Suzanne Shusterman, MD 617-632-4901
STEM CELL TRANSPLANTATION			
Stem Cell Transplantation (Severe Aplastic Anemia)	Fludarabine-Based Conditioning for Allogeneic Marrow Transplantation From HLA-Compatible Unrelated Donors in Severe Aplastic Anemia (CTN 0301)	06-158/I-II	Leslie Lehmann, MD 617-632-4882
Stem Cell Transplantation (Leukemia and Myelodysplasia)	Multi-Center, Open Label, Randomized Trial Comparing Single Versus Double Umbilical Cord Blood (UCB) Transplantation in Pediatric Patients with Leukemia and Myelodysplasia	07-031/III	Leslie Lehmann, MD 617-632-4882
Stem Cell Transplantation (ALL)	A Randomized Trial of Sirolimus-Base Graft versus Host Disease Prophylaxis after Hematopoietic Stem Cell Transplantation in Selected Patients with CR1 and CR2 Acute Lymphoblastic Leukemia (ALL)	07-368/III	Leslie Lehmann, MD 617-632-4882

STEM CELL TRANSPLANTATION			
Stem Cell Transplantation (Sickle Cell Disease)	Unrelated Donor Hematopoietic Stem Cell Transplantation for Children with Severe Sickle Cell Disease Using a Reduced Intensity Conditioning Regimen	09-141/II	Leslie Lehmann, MD 617-632-4882
Stem Cell Transplantation (Severe Thalassemia)	Unrelated Donor Hematopoietic Stem Cell Transplantation for Children with Severe Thalassemia Using Reduced Intensity Conditioning Regimen (The URTM Trial)	09-458/II	Leslie Lehmann, MD 617-632-4882
Stem Cell Transplantation (Severe Hepatic Venous-Occlusive Disease)	Defibrotide for Hematopoietic Stem Cell Transplant Patients with Severe Hepatic Venous-Occlusive Disease (VOD): A Treatment IND Study (UnderCFR312.34)	07-320/III	Paul Richardson, MD 617-632-2104
Stem Cell Transplantation (SCID)	Gene Transfer for SCID-X1 using a self-inactivating (SIN) gammaretroviral vector	09-05-0254	Sung-Yun Pai, MD 617-632-3956
OTHER			
Hemophagocytic lymphohistiocytosis (HLH)	HLH- 2004 – Treatment Protocol of the 2nd International HLH Study	05-137/Pilot	Barbara Degar, MD 617-632-6801
Neurofibromatosis, plexiform neurofibromas	Phase II Study of mTOR Inhibitor Sirolimus for Plexiform Neurofibromas	08-035/II	Nicole Ullrich, MD, PhD 617-355-2066
Neurofibromatosis, 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
Neurofibromatosis	A Randomized Placebo-Controlled Study of Lovastatin in Children with Neurofibromatosis Type I (for Children with Cognitive and Attention Problems)	09-05-0228	Nicole Ullrich, MD, PhD 617-355-2066
Supportive Care	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	08-339/I	Peter Manley, MD 617-632-4368

Dana-Farber/Children's Hospital Cancer Center 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, the Transfusion Medicine Hemostasis Clinical Research Network and, Therapeutic Advances in Childhood Leukemia and Lymphoma (TACL).

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 Center 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.