



## DANA-FARBER/CHILDREN'S HOSPITAL CANCER CENTER

## **Selected Protocols for the Treatment of Pediatric Cancer and Blood Disorders**

This listing changes frequently. For more information about the trials we have available please contact dfchcc\_clinicaltrials@dfci.harvard.edu

| Treatment Area  | Study Name  | ID Number/<br>Phase | Site Investigator                       |  |  |
|---|---|---------------------|---|--|--|
| BRAIN TUMORS  | BRAIN TUMORS  |                     |   |  |  |
| Diffuse Intrinsic Pontine<br>Glioma (DIPG)                          | Phase II Trial of Molecularly Determined Treatment of Children<br>and Young Adults with Newly Diagnosed Diffuse Intrinsic<br>Pontine Glioma   | 10-321/II           | Mark W. Kieran, MD, PhD<br>617-632-4386 |  |  |
| Ependymoma  | A Phase II Multi-Institutional Trial of Focal Radiotherapy<br>with Concomitant Carboplatin as a Radiosensitizer and the<br>Prospective Analysis of Survivin, an Inhibitor of Apoptosis, as a<br>Biomarker in Children with Newly Diagnosed Non-Metastatic<br>Ependymoma and Minimal Residual Disease Post-Operatively   | 10-395/II           | Susan Chi, MD<br>617-632-4386           |  |  |
| Ependymoma<br>(Recurrent)   | Erlotinib vs Etoposide for Recurrent Ependymoma   | 10-093/II           | Mark W. Kieran, MD, PhD<br>617-632-4386 |  |  |
| 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<br>617-632-4386 |  |  |
| High Grade Glioma and<br>Diffuse Intrinsic Pontine<br>Glioma (DIPG) | Cetuximab, Irinotecan and XRT for Newly Diagnosed HGG and Diffuse Intrinsic Pontine Glioma  | 09-123/II           | Mark W. Kieran, MD, PhD<br>617-632-4386 |  |  |
| Low Grade Glioma and<br>Neurofibromatosis type 1                    | A Phase II Study of RAD001 (Everolimus) for Children with<br>Neurofibromatosis type I and Chemotherapy-Refractory<br>Progressive or Recurrent Low-Grade Gliomas (DOD)   | 09-452/II           | Mark W. Kieran, MD, PhD<br>617-632-4386 |  |  |
| Recurrent Brain Tumors  | A phase I study of LDE225 in Pediatric patients with recurrent or refractory Medulloblastoma, or other tumors potentially dependent on Hedgehog signaling pathway   | 10-358/I            | Mark W. Kieran, MD, PhD<br>617-632-4386 |  |  |
| BENIGN HEMATOLOGY   |   |                     |   |  |  |
| Sickle Cell Disease   | Stroke With Transfusions Changing to Hydroxyurea (SWiTCH): A Phase III 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          | Matthew Heeney, MD<br>617-355-7700      |  |  |
| Fanconi Anemia/<br>Dyskeratosis Congenita                           | Phase I/II Dose Escalation Trial of Danazol in Patients with Fanconi Anemia or Dyskeratosis Congenita   | 09-03-0131/I-II     | Colin Sieff, MB BCh<br>617-919-4241     |  |  |

| Treatment Area  | Study Name   | ID Number/<br>Phase          | Site Investigator                      |
|---|--|------------------------------|--|
| BENIGN HEMATOLOGY (co   | ontinued)  |                              |  |
| Hemophilia  | Pediatric Phase IIIb Trial of rFVIII (N8) in Patients with Hemophilia A  | P00001174/III                | Kapil Saxena, MD<br>617-355-4977       |
| Hemophilia  | Bleeding in Surgical Interventions or Invasive Procedures in<br>Hemophilia A or B Patients with Inhibitors to Factor FVIII or<br>Factor IX   | P00001088/III                | Kapil Saxena, MD<br>617-355-4977       |
| Idiopathic<br>Thrombocytopenic Purpura                        | A Three-Part, Staggered Cohort, Open-Label and Double-Blind,<br>Randomized, Placebo Controlled Study to Investigate the<br>Efficacy, Safety, Tolerability and Pharmacokinetics of Eltrom-<br>bopag, a Thrombopoietin Receptor Agonist, in Previously<br>Treated Pediatric Patients with Chronic Idiopathic Thrombocyto-<br>penic Purpura (ITP) | 10-02-0056/III               | Ellis Neufeld, MD, PhD<br>617-919-2139 |
| Idiopathic<br>Thrombocytopenic Purpura                        | Initial Treatment of Patients with Immune Thrombocytopenic<br>Purpura  | 10-030                       | Ellis Neufeld, MD, PhD<br>617-919-2139 |
| Iron Overload   | A Phase 2, Open Label, Multi-Center Single-Dose Pharmacoki-<br>netics, and Multiple Dose Study of the Safety, Efficacy and<br>Tolerability of FBS0701 in Pediatric Population with Transfu-<br>sional Iron Overload  | P00000983                    | Rachel Grace, MD<br>617-919-2144       |
| Myelodysplastic Syndrome/<br>Bone Marrow Failure<br>Disorders | Pediatric Myelodysplastic Syndrome and Bone Marrow Failure<br>Disorder Patient Registry and Tissue Repository  | 10-02-0057                   | Inga Hofmann, MD<br>617-355-2463       |
| Severe Combined Immuno-<br>deficiency Syndrome (SCID)         | Gene Transfer for SCID-X1 Using a Self-Activating (SIN) Gammaretroviral Vector   | 09-05-0254                   | Luigi Notarangelo MD<br>617-919-2276   |
| Sickle Cell Disease   | Safety of Adenosine 2A Agonist Lexiscan in Children and Adults with Sickle Cell Disease  | 09-308/I                     | Matthew Heeney, MD<br>617-355-7700     |
| Sickle Cell Disease   | Sickle Cell Study of Prasurgel   | P00001064                    | Matthew Heeney, MD<br>617-355-7700     |
| Sickle Cell Disease   | TCD with Transfusions Changing to Hydroxyurea  | P00001496/III                | Matthew Heeney, MD<br>617-355-7700     |
| Thalassemia   | Health-Related Quality of Life in Patients Requiring Chronic Red<br>Cell Transfusions: Validation Study (TranQOL)  | X10-05-0249                  | Ellis Neufeld, MD, PhD<br>617-919-2139 |
| 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/<br>IV           | Ellis Neufeld, MD, PhD<br>617-919-2139 |
| Thalassemia   | Thalassemia Data and Blood Specimen Collection System  | 03-10-163                    | Ellis Neufeld, MD, PhD<br>617-919-2139 |
| Thalassemia   | Thalassemia Longitudinal Cohort<br>(Accrual Complete Except for Patients Undergoing Transplant)  | 06-10-0466/<br>Observational | Ellis Neufeld, MD, PhD<br>617-919-2139 |

| Treatment Area  | Study Name  | ID Number/<br>Phase | Site Investigator                            |
|---|---|---------------------|--|
| Vascular Anomalies                                      | Phase II clinical trial assesing efficacy and safety of the mTOR Inhibitor Sirolimus in the treatment of complicated vascular anomalies   | P00001573/II        | Cameron Trenor, MD<br>(617) 919-3242         |
| Wiskott-Aldrich Syndrome                                | Pilot and feasibility study of hematopoietic stem cell gene transfer for the Wiskott-Aldrich Syndrome   | P00000148           | Sung-Yun Pai, MD<br>617-919-2508             |
| DEVELOPMENTAL THERAF                                    | PEUTICS   |                     |  |
| Solid Tumor<br>(incl., Lymphoma)<br>relapsed/refractory | A Phase I Study of Ipilimumab (Anti-CTLA-4) in Children, Adolescents, and Young Adults with Treatment Refractory Cancer   | 11-062//I           | Carlos Rodriguez-Galindo, MD<br>617-632-4580 |
| Solid Tumor,<br>relapsed/refractory                     | A Phase 1/2 Study of EZN-2208 in Children, Adolescents, and<br>Young Adults with Relapsed or Refractory Solid Tumors  | 10-010/I-II         | Suzanne Shusterman, MD<br>617-632-4901       |
| Solid Tumor,<br>relapsed/refractory                     | Individualized Cancer Therapy (iCat) Recommendation for Patients with Recurrent, Refractory, or High Risk Solid Tumors  | 11-406              | Katherine Janeway, MD<br>617-632-4994        |
| Solid Tumor,<br>relapsed/refractory                     | Phase I Dose Escalation Study of Sorafenib and Irinotecan<br>Combination Therapy in Pediatric Patients with Relapsed or<br>Refractory Solid Tumors  | 11-420/I            | Carlos Rodriguez-Galindo, MD<br>617-632-4580 |
| EMBRYONAL TUMORS  |   |                     |  |
| Neuroblastoma<br>(newly diagnosed)                      | A Comparative Pharmacokinetic and Safety Study of Chimeric<br>Monoclonal Antibody ch 14.18 with Granulocyte-Macrophage-<br>Colony-Stimulating-Factor (GM-CSF), Interleukin (IL-2) and<br>Isotretinoin in high-Risk Neuroblastoma Patients Following<br>Myeloblative Therapy | 12-141              | Suzanne Shusterman, MD<br>617-632-4901       |
| Neuroblastoma,<br>relapsed/refractory                   | Phase I Study of Fenretinide (4-HPR, NSC 374551) Lym-X-<br>sorb™(LXS) Oral Powder in Patients with Recurrent or Resistant<br>Neuroblastoma (NANT 04-04)   | 05-302/I            | Suzanne Shusterman, MD<br>617-632-4901       |
| Neuroblastoma,<br>relapsed/refractory                   | An Open Label, Expanded Access Protocol Using 131I-Metaiodobenzylguanidine (131 I-MIBG) Therapy in Patients with Refractory Neuroblastoma   | 08-111/I            | Suzanne Shusterman, MD<br>617-632-4901       |
| Neuroblastoma,<br>relapsed/refractory                   | A Phase I Study of Bevacizumab with Bolus and Metronomic<br>Cyclophosphamide and Zoledronic Acid in Children with Recur-<br>rent or Refractory Neuroblastoma (NANT 07-02)   | 09-078/I            | Suzanne Shusterman, MD<br>617-632-4901       |
| Neuroblastoma,<br>relapsed/refractory                   | Modulation of Intensive Melphalan (L-PAM) by Buthionine Sulf-<br>oximine (BSO) with Autologous Stem Cell Support for Resistant/<br>Recurrent High-Risk Neuroblastoma (NANT99-02)  | 09-225/I            | Suzanne Shusterman, MD<br>617-632-4901       |
| Neuroblastoma,<br>relapsed/refractory                   | Vorinostat +131 I-MIBG for Relapsed Neuroblastoma (NANT N2007-03)   | 10-051/I            | Suzanne Shusterman, MD<br>617-632-4901       |
| Neuroblastoma,<br>relapsed/refractory                   | Phase I Study Of Vorinostat In Combination With 13-Cis-Retinoic<br>Acid In Patients With Refractory/Recurrent Neuroblastoma<br>(NANT 2008-02)   | 10-408/I            | Suzanne Shusterman, MD<br>617-632-4901       |
| Neuroblastoma,<br>relapsed/refractory                   | A Phase I Trial Combining Decitabine and Vaccine Therapy for Patients with Neuroblastoma  | 11-130/I            | Rani George, MD<br>617-632-5281              |

| Treatment Area   | Study Name   | ID Number/<br>Phase | Site Investigator                            |
|--|--|---------------------|--|
| HEMATOLOGIC MALIGNA                                    | NCY  |                     |  |
| Langerhans Cell Histiocytosis<br>(relapsed/refractory) | Phase II Study of Clofarabine in Patients with Recurrent or<br>Refractory Langerhans Cell Histiocytosis  | 12-050              | Carlos Rodriguez-Galindo, MD<br>617-632-4580 |
| Leukemia (ALL/AML)<br>Relapsed/refractory              | AC 220 for Children with relapsed ALL or AML   | 12-076              | Lewis Silverman, MD<br>617-632-6191          |
| Hemophagocytic<br>lymphohistiocytosis (HLH)            | An open label study of Hybrid ImmunoTherapy (ATG/Dexameth-<br>asone/Etoposide) for Hemophagocytic LymphoHistiocytosis:<br>HIT-HLH  | 11-243/II           | Barbara Degar, MD<br>617-632-5186            |
| Hodgkins Lymphoma<br>(high risk)                       | Reduced Duration Stanford v Chemotherapy with Low-Dose<br>Tailored-Field Radiation Therapy for Favorable Risk Pediatric<br>Hodgkin Lymphoma (HOD08)  | 09-273/II           | Amy Billett, MD<br>617-632-5640              |
| Leukemia (ALL)<br>newly diagnosed                      | Randomized Study of Intravenous Calaspargase Pegol (SC-PEG asparaginase) and Intravenous Oncaspar in Children and Adolescents with Acute Lymphoblastic Leukemia or Lymphoblastic Lymphoma.   | 11-001/III          | Lewis Silverman, MD<br>617-632-6191          |
| Leukemia (Infant ALL),<br>newly diagnosed              | Interfant-06 — International Collaborative Treatment Protocol<br>for Infants under One Year with Acute Lymphoblastic Leukemia<br>or Biphenotypic Leukemia  | 06-172/III          | Lewis Silverman, MD<br>617-632-6191          |
| Leukemia (ALL),<br>relapsed/refractory                 | A Feasibility trial of Everolimus (RAD001), an mTOR inhibitor, given in combination with multiagent re-induction chemotherapy in pediatric patients with relapsed acute lymphoblastic Leukemia (ALL)   | 11-237/I            | Lewis Silverman, MD<br>617-632-6191          |
| Leukemia (ALL),<br>relapsed/refractory                 | Clofarabine with Cytarabine for MRD Positive Leukemia  | 11-338/II           | Lewis Silverman, MD<br>617-632-6191          |
| Leukemia (ALL/AML),<br>relapsed/refractory             | A Phase I, Open-Label, Dose-Escalating Study to Evaluate<br>the Safety, Tolerability and Pharmacokinetics of Twice Daily<br>Oral Midostaurin and to Evaluate the Preliminary Clinical and<br>Phamacodynamic Response in Pediatric Patients with Relapsed<br>or Refractory Leukemia | 09-238/I            | Lewis Silverman, MD<br>617-632-6191          |
| Leukemia (ALL),<br>relapsed/refractory                 | A Phase I Trial of CAT-8015 (Toxin-conjugated anti-CD22) for<br>Relapsed, Refractory B-precursor ALL   | 09-340/I            | Lewis Silverman, MD<br>617-632-6191          |
| Leukemia (ALL),<br>relapsed/refractory                 | A Phase I Trial of Nelarabine, Cyclophosphamide, Etoposide in<br>Relapsed, Refractory T-Cell ALL   | 10-052/I            | Lewis Silverman, MD<br>617-632-6191          |
| Leukemia (AML)   | 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<br>617-632-5186            |
| Leukemia (CML and Ph+<br>leukemias)                    | A Phase II Study of Dasatinib Therapy in Children and<br>Adolescents with Newly Diagnosed Chronic Phase Chronic<br>Myelogenous Leukemia or with Ph+ Leukemias Resistant or<br>Intolerant to Imatinib   | 11-084/II           | Barbara Degar, MD<br>617-632-5186            |

| Treatment Area  | Study Name  | ID Number/<br>Phase | Site Investigator                            |
|---|---|---------------------|--|
| RARE AND ADULT CANCER   | S   |                     |  |
| Gastrointestinal Stromal<br>Tumor                                       | A Phase I/II Study of Sunitinib in Young Patients with Advanced<br>Gastrointestinal Stromal Tumor   | 12-106              | Katherine Janeway, MD<br>617-632-4994        |
| Melanoma  | An open-label, multicenter, single-arm, Phase I dose-escalation with efficacy tail extension study of RO5185426 in pediatric patients with surgically incurable and unresectable Stage IIIC or Stage IV melanoma harboring BRAFV600 mutations               | 11-388/I            | Carlos Rodriguez-Galindo, MD<br>617-632-4580 |
| SARCOMA   |   |                     |  |
| Osteosarcoma,<br>relapsed/refractory                                    | A Randomized, Double-Blinded, Placebo-Controlled, Multi-Institutional, Phase II.5 Study of AZD0530, a Selective SRC Kinase Inhibitor, in Patients with Recurrent Osteosarcoma Localized to the Lung   | 09-473/II-III       | Katherine Janeway, MD<br>617-632-4994        |
| STEM CELL TRANSPLANTA   | TION  |                     |  |
| Stem Cell Transplantation<br>(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<br>617-919-4610           |
| Stem Cell Transplantation<br>(Severe Aplastic Anemia)                   | Fludarabine-Based Conditioning for Allogeneic Marrow Trans-<br>plantation From HLA-Compatible Unrelated Donors in Severe<br>Aplastic Anemia (CTN 0301)  | 06-158/I-II         | Leslie Lehmann, MD<br>617-632-4882           |
| Stem Cell Transplantation<br>(Fanconi Anemia)                           | 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, Cyclophosphasmide and Fludarabine | 08-377/II           | David A. Williams, MD<br>617-919-2697        |
| Stem Cell Transplantation<br>(Cord Blood Unit access)                   | A Multicenter Access and Distribution Protocol for Unlicensed<br>Cryopreserved Cord Blood Units (CBUs) for Transplantation in<br>Pediatric and Adult Patients with Hematologic Malignancies and<br>Other Indications  | 11-297              | Leslie Lehmann, MD<br>617-632-4882           |
| Stem Cell Transplantation<br>(Graft-Versus-Host Disease)                | An Open Label Phase II Randomized trial of Topical Dexamethasone and Tacrolimus for the Treatment of Oral Chronic Graft-Versus-Host Disease   | 08-027/II           | Nathaniel S. Treister, DMD<br>617-632-2806   |
| Stem Cell Transplantation<br>(Sickle Cell Disease)                      | Unrelated Donor Hematopoietic Stem Cell Transplantation for<br>Children with Severe Sickle Cell Disease Using a Reduced Inten-<br>sity Conditioning Regimen   | 09-141/II           | Leslie Lehmann, MD<br>617-632-4882           |
| Stem Cell Transplantation<br>(Severe Thalassemia)                       | Unrelated Donor Hematopoietic Stem Cell Transplantation for<br>Children with Severe Thalassemia Using Reduced Intensity<br>Conditioning Regimen (The URTH Trial)  | 09-458/II           | Leslie Lehmann, MD<br>617-632-4882           |
| Stem Cell Transplantation<br>(Severe Hepatic<br>Veno-Occlusive Disease) | Defibrotide for Hematopoietic Stem Cell Transplant Patients with Severe Hepatic Veno-Occlusive Disease (VOD): A Treatment IND Study (UnderCFR312.34)  | 07-320/III          | Leslie Lehmann, MD<br>617-632-4882           |
| Stem Cell Transplantation (SCID)  | Gene Transfer for SCID-X1 using a self-inactivating (SIN) gammaretroviral vector  | 09-05-0254          | Luigi Notarangelo, MD<br>617-919-2276        |
| Wiskott-Aldrich Syndrome  | Pilot and feasibility study of hematopoietic stem cell gene trans-<br>fer for the Wiskott-Aldrich Syndrome  | P00000148           | Sung-Yun Pai, MD<br>617-919-2508             |

| Treatment Area                                | Study Name   | ID Number/<br>Phase | Site Investigator                       |
|---|--|---------------------|---|
| OTHER   |  |                     |   |
| Solid Tumor<br>(relapsed/refractory)          | A Phase I/II Clinical Study of Vorinostat in Combination with<br>Etoposide in Pediatric Patients <21 Years at Diagnosis with<br>Refractory Solid Tumors          | 12-003              | Mark W. Kieran, MD, PhD<br>617-632-4368 |
| Solid Tumor<br>(Lung metasteses)              | Cardiac-Sparing Whole-Lung IMRT in Children and Young Adults with Lung Metastases: a Feasability Study   | 10-463              | Karen Marcus, MD<br>617-525-7386        |
| Neurofibromatosis,<br>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<br>617-632-4368 |
| Neurofibromatosis                             | A Randomized Placebo-Controlled Study of Lovastin in Children with Neurofibromatosis Type I (for Children with Cognitive and Attention Problems)                 | 09-05-0228          | Nicole Ullrich, MD, PhD<br>617-355-2066 |

## Children's Oncology Group (COG) trials open at Dana-Farber/Children's Hospital Cancer Center

| Treatment Area                               | Study Name   | ID Number/<br>Phase | Site Investigator               |
|--|--|---------------------|---------------------------------|
| BRAIN TUMOR                                  |  |                     |                                 |
| Atypical Teratoid/Rhabdoid<br>Tumors (AT/RT) | Treatment of Atypical Teratoid/Rhabdoid Tumors (AT/RT) of the<br>Central Nervous System with Surgery, Intensive Chemotherapy,<br>and 3-D Conformal Radiation (ACNS0333)  | 11-092/III          | Susan Chi, MD<br>617-632-4386   |
| Diffuse Intrinsic Pontine<br>Glioma (DIPG)   | A Phase I/II Study of suberoylanilide hydroxamic acid (SAHA,<br>Vorinistat and Local Irradiation followed by Maintenance SAHA<br>in Children with Newly Diagnosed Diffuse Intrinsic Pontine<br>Gliomas (DIPG) (ACNS0927)   | 11-032/I            | Susan Chi, MD<br>617-632-4386   |
| Ependymoma                                   | Phase III Randomized Trial of Post-Radiation Chemotherapy<br>in Patients with Newly Diagnosed Ependymoma Ages 1 to 21<br>years (ACNS0831)  | 10-239/III          | Susan Chi, MD<br>617-632-4386   |
| Medulloblastoma                              | A Phase III Study Investigating the Efficacy of Carboplatin<br>Administered Concomitantly with Radiation and Isotretinoin as<br>a Pro-Apoptotic Agent in Other than Average Risk Medulloblas-<br>toma/PNET Patients (COG ACNS0332)                                 | 07-125/III          | Susan Chi, MD<br>617-632-4386   |
| Medulloblastoma<br>(infants/toddlers)        | Randomized Phase III Study for Patients with High Risk PNET/<br>Medulloblastoma under the Age of 36 Months (COG ACNS0334)  | 07-308/III          | Susan Chi, MD<br>617-632-4386   |
| Medulloblastoma<br>(Recurrent/Refractory)    | Temozolomide with Irinotecan versus Temozolomide, Irinotecan plus Bevacizumab for Recurrent/Refractory Medulloblastoma/CNS PNET of Childhood, A COG Randomized Phase II Screening Trial (ACNS0821)   | 11-150/II           | Susan Chi, M.D.<br>617-632-4386 |
| High Grade Glioma                            | A Randomized Phase II/III Study of Vorinostat and Local Irradiation OR Temozolomide and Local Irradiation OR Bevacizumab and Local Irradiation Followed by Maintenance Bevacizumab and Temozolomide in Children with Newly Diagnosed High-Grade Gliomas (ACNS0822) | 11-079/II-III       | Susan Chi, MD<br>617-632-4386   |

| Treatment Area   | Study Name   | ID Number/<br>Phase | Site Investigator                            |  |
|--|--|---------------------|--|--|
| DEVELOPMENTAL THERAF                                     | PEUTICS  |                     |  |  |
| Solid Tumor<br>(relapsed/refractory)                     | Phase II Study of the Raf Kinase and Receptor Tyrosine Kinase<br>Inhibitor Sorafenib (IND# 69896) in Children and Young Adults<br>with Relapsed/Refractory Rhabdomyosarcoma, Wilms Tumor,<br>Hepatocellular Carcinoma, and Papillary Thyroid Carcinoma<br>(ADVL1121)           | 12-046              | Carlos Rodriguez-Galindo, MD<br>617-632-4580 |  |
| Solid Tumor,<br>relapsed/refractory                      | A Phase I Dose Escalation Study of Seneca Valley Virus (SVV-<br>001), a Replication-Competent Picornavirus, in Relapsed/<br>Refractory Pediatric Patients with Neuroblastoma, Rhabdo-<br>myosarcoma, Ewing Family of Tumors, and Rare Tumors with<br>Neuorendocrine (ADVL0911) | 10-243/I            | Suzanne Shusterman, MD<br>617-632-4901       |  |
| Solid Tumor,<br>relapsed/refractory                      | A Phase 1 Study of Temsirolimus in Combination with Irirno-<br>tecan and Temozolomide in Children, Adolescents, and Young<br>Adults with Relapsed or Refractory Solid Tumors (ADVL0918)  | 10-248/I            | Suzanne Shusterman, MD<br>617-632-4901       |  |
| Solid Tumor,<br>(incl., Leukemia)<br>relapsed/refractory | A Phase 1 Study of MK-2206, an AKT inhibitor, in Pediatric<br>Patients with Recurrent or Refractory Solid Tumors or Leukemia<br>(ADVL1013)   | 11-058/I            | Suzanne Shusterman, MD<br>617-632-4901       |  |
| Solid Tumor,<br>(incl., Leukemia)<br>relapsed/refractory | A Phase II Study of MLN8237 (IND# 102984), a Selective Aurora<br>A Kinase Inhibitor in Children with Recurrent/Refractory Solid<br>Tumors and Leukemias ADVL0921)  | 11-102/II           | Suzanne Shusterman, MD<br>617-632-4901       |  |
| Solid Tumor (incl, Lympho-<br>ma), relapsed/refractory   | A Phase 1 Study of Imetelstat, a Telomerase Inhibitor, in Children with Refractory or Recurrent Solid Tumors and Lymphoma (ADVL1112)   | 11-183/I            | Suzanne Shusterman, MD<br>617-632-4901       |  |
| Solid Tumor (incl., Lympho-<br>ma), relapsed/refractory  | A Phase I/II Study of PF-02341066, an Oral Small Molecule<br>Inhibitor of Anaplastic Lymphoma Kinase (ALK) and C-Met, in<br>Children with Relapsed/Refractory Solid Tumors and Anaplastic<br>Large Cell Lymphoma (COG ADVL0912)  | 09-347/I-II         | Suzanne Shusterman, MD<br>617-632-4901       |  |
| Solid Tumor,<br>relapsed/recurrent                       | A Phase I Study of Irinotecan, Temozolomide and Temsirolimus in Children with Refractory or Recurrent Solid Tumors (ADVL0918)  | 10-248/I            | Suzanne Shusterman, MD<br>617-632-4901       |  |
| Solid Tumor (Leukemia),<br>relapsed/refractory           | Phase 1 Study of JAK Inhibition (INCB018424) in Children with<br>Relapsed or Refractory Solid Tumors, Leukemias, and Myelopro-<br>liferative Neoplasms (ADVL1011)  | 10-350/I            | Allison O'Neill, MD<br>617-632-4202          |  |
| EMBRYONAL TUMORS   |  |                     |  |  |
| Hepatoblastoma   | Treatment of Children with All Stages of Hepatoblastoma<br>(COG identifier (AHEP 0731)   | 09-379/III          | Suzanne Shusterman, MD<br>617-632-4901       |  |
| Neuroblastoma, OMS                                       | Use of Intravenous Gammaglobulin Therapy for Patients with<br>Neuroblastoma Associated Opsoclonus-Myoclonus-Ataxia Syn-<br>drome Treated with Chemotherapy and Prednisone (ANBL00P3)   | 06-184/III          | Suzanne Shusterman, MD<br>617-632-4901       |  |
| Neuroblastoma,<br>high risk                              | Phase III Randomized Trial of Single versus Tandem Myelobla-<br>tive as Consolidation Therapy for High-Risk Neuroblastoma<br>(ANBL0532)  | 07-403/III          | Suzanne Shusterman,MD<br>617-632-4901        |  |

| Treatment Area                            | Study Name   | ID Number/<br>Phase | Site Investigator                            |
|---|--|---------------------|--|
| EMBRYONAL TUMORS (co                      | ntinued)   |                     |  |
| Neuroblastoma,<br>relapsed/refractory     | Feasibility/Phase II Study of hu14.18-IL2 immunocytokine + GM-CSF and Isotretinoin in Patients with Relapsed or Refractory Neuroblastoma (ANBL1021)  | 11-393/II           | Elizabeth Mullen, MD<br>617-632-5186         |
| Renal Tumors,high risk                    | Treatment of High Risk Renal Tumors (AREW0321)   | 06-263/III          | Elizabeth Mullen, MD<br>617-632-5186         |
| Wilms Tumor, low risk                     | Treatment for Very Low and Standard Risk Favorable Histology Wilms Tumor (AREW0532)  | 07-008/III          | Elizabeth Mullen, MD<br>617-632-5186         |
| Wilms Tumor, high risk                    | Treatment of Newly Diagnosed Higher Risk Favorable Histology<br>Wilms Tumors (AREW0533)  | 07-095/III          | Elizabeth Mullen, MD<br>617-632-5186         |
| Wilms Tumor                               | Treatment for Patients with Bilateral, Multicentric, or Bilaterally-<br>Predisposed Unilateral Wilms Tumor (AREW0534)  | 11-476/III          | Elizabeth Mullen, MD<br>617-632-5186         |
| HEMATOLOGIC MALIGNA                       | NCIES  |                     |  |
| Leukemia<br>(newly diagnosed)             | A Phase 2 Multi-Center, Historically-Controlled Study of Dasatinib Added to Standard Chemotherapy in Pediatric Patients with Newly Diagnosed Philadelphia Chromosome Positive Acute Lymphoblastic Leukemia (AALL1122)                                      | 12-056              | Lewis Silverman, MD<br>617-632-6191          |
| Leukemia (APL),<br>relapsed/refractory    | Risk Adapted Treatment of Newly Diagnosed Childhood Acute<br>Promyelocytic Leukemia (APL) using Arsenic Trioxide (Trisenox,<br>IND#103,331) during Consolidation (AAML0631)  | 10-112/III          | Barbara Degar, MD<br>617-632-5186            |
| RARE AND ADULT CANCE                      | RS   |                     |  |
| Adrenocortical Tumors                     | Treatment of Adrenocortical Tumors with Surgery plus Lymph<br>Node Dissection and Multiagent Chemotherapy (ARAR0332  | 07-180/III          | Carlos Rodriguez-Galindo, MD<br>617-632-4580 |
| SARCOMA                                   |  |                     |  |
| Ewing Sarcoma,<br>non-metastatic          | A Phase III Randomized Trial of Adding Vincristine-topotecan-cy-<br>clophosphamide to Standard Chemotherapy in Initial Treatment<br>of Non-metastatic Ewing Sarcoma: A Groupwide Phase III Study<br>(AEWS1031)   | 10-427/III          | Katherine Janeway, MD<br>617-632-4994        |
| Rhabdomyosarcoma, intermediate risk       | Randomized Study of Vincristine, Dactinomycin and Cyclophos-<br>phamide (VAC) versus VAC Alternating with Vincristine and<br>Irinotecan (VI) for Patients with Intermediate-Risk RMS (RMS)<br>(ARST0531)   | 07-030/III          | Carlos Rodriguez-Galindo, MD<br>617-632-4580 |
| Rhabdomyosarcoma,<br>high risk            | A Pilot Study to Evaluate Novel Agents (Temozolomide and Cixutumumab [IMC-A12, Anti-IGF-IR Monoclonal Antibody, IND# 100947, NSC#742460]) in Combination with Intensive Multi-Agent Interval Compressed Therapy for Patients with High-Risk RMS (ARST08P1) | 10-124/ Pilot       | Carlos Rodriguez-Galindo, MD<br>617-632-4580 |
| Rhabdomyosarcoma,,<br>relapsed/refractory | A Randomized Phase II Trial of Bevacizumab (IND# 7921, Avastin) and Temsirolimus (IND# 61010, Torisel) in Combination with Intravenous Vinorelbine and Cyclophosphamide in Patients with Recurrent/Refractory Rhabdomyosarcoma (COG ARST0921)              | 10-413/II           | Carlos Rodriguez-Galindo, MD<br>617-632-4580 |

| Treatment Area                           | Study Name   | ID Number/<br>Phase | Site Investigator                    |
|--|--|---------------------|--------------------------------------|
| STEM CELL TRANSPLANTA                    | TION   |                     |                                      |
| Stem Cell Transplantation<br>(Mucositis) | A Randomized Double Blinded Trial of Topical Caphosol to Prevent Oral Mucositis in Children Undergoing Hematopoietic Stem Cell Transplantation (ACCL1031)                                | 11-301              | Christine Duncan, MD<br>617-632-4882 |
| Stem Cell Transplantation<br>(Other)     | A Randomized Trial of Levofloxacin to Prevent Bacteremia in<br>Children Being Treated for Acute Leukemia (AL) or Undergoing<br>Hematopoietic Stem Cell Transplantation (HSCT) (ACCL0934) | 11-384              | Christine Duncan, MD<br>617-632-4882 |

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 blood diseases.