

Names Family Foundation Early Phase Clinical Trials Program Phase I and Phase II Clinical Trials: Hematology, Oncology, and Blood & Marrow Transplantation

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ONCOLOGY

Solid Tumors				
NCT Number	Title	Sponsor	Investigator	Contact
NCT02637687 <i>Phase I</i>	A Phase I Study of the Oral TRK Inhibitor LOXO-101 in Pediatric Patients with Advanced Solid or Primary Central Nervous System Tumors	LOXO Oncology	Leo Mascarenhas, MD	Hemlata Sukhija hsukhija@chla.usc.edu
NCT02044120 <i>Phase I</i>	ESP1/SARC025 Global Collaboration: A Phase I Study of a Combination of the PARP Inhibitor, Niraparib and Temozolomide or Irinotecan in Patients with Previously Treated, Incurable Ewing Sarcoma	SARC	Leo Mascarenhas, MD	Hemlata Sukhija hsukhija@chla.usc.edu
NCT02095132 <i>Phase I/II</i>	ADVL1312: A Phase I/II Study of AZD1775 (MK-1775, IND# 121422) in Combination with Oral Irinotecan in Children, Adolescents, and Young Adults with Relapsed or Refractory Solid Tumors	COG	Leo Mascarenhas, MD	Hemlata Sukhija hsukhija@chla.usc.edu
NCT01606878 <i>Phase I</i>	ADVL1212 A Phase I Study of Crizotinib (IND#105573) in Combination with Conventional Chemotherapy for Relapsed or Refractory Solid Tumors or Anaplastic Large Cell Lymphoma	COG	Leo Mascarenhas, MD	Hemlata Sukhija hsukhija@chla.usc.edu
NCT02116777 <i>Phase I/II</i>	ADVL1411: A Phase I/II Study of BMN 673 (IND# 121510), an Oral Poly (ADP-RIBOSE) Polymerase Inhibitor, Plus Temozolomide in Children with Refractory or Recurrent Malignancies	COG	Leo Mascarenhas, MD	Hemlata Sukhija hsukhija@chla.usc.edu
NCT02304458 <i>Phase I/II</i>	ADVL1412: A Phase I/II 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	COG	Leo Mascarenhas, MD	Hemlata Sukhija hsukhija@chla.usc.edu

NCT01956669 <i>Phase II</i>	ADVL1322 A Phase II Trial of Pazopanib in Children with Refractory Solid Tumors	COG/ Novartis	Leo Mascarenhas, MD	Hemlata Sukhija hsukhija@chla.usc.edu
NCT02180867 <i>Phase II/III</i>	ARST1321 A Phase II/III Randomized Trial of Preoperative Chemoradiation or Preoperative Radiation Plus or Minus Pazopanib (NSC #737754, IND#118613)	COG	Leo Mascarenhas, MD	Hemlata Sukhija hsukhija@chla.usc.edu
NCT02470091 <i>Phase II</i>	AOST1321 A Phase II Study of Denosumab (IND#127430, NSC#744010), a RANK Ligand Antibody, for Recurrent or Refractory Osteosarcoma	COG	Leo Mascarenhas, MD	Magnolia Manzano mmanzano@chla.usc.edu
NCT01767194 <i>Phase II</i>	ANBL1221 A Phase II Randomized Trial of Irinotecan/Temozolomide with Temsirolimus (NSC#683864, IND#61010) or Chimeric 14.18 Antibody in Children with Refractory, Relapsed or Progressive Neuroblastoma	COG	Araz Marachelian, MD	Elysia Kim elykim@chla.usc.edu
NCT02945800 <i>Phase II</i>	MCC-18613: Phase II Study of nab-Paclitaxel in Combination with Gemcitabine for Treatment of Recurrent/Refractory Sarcoma in Teenagers and Young Adults	Sunshine Project-PCF/Moffitt Cancer Center	Leo Mascarenhas, MD	Magnolia Manzano mmanzano@chla.usc.edu
NCT02332668 <i>Phase I/II</i>	ADVL1621 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	Merck Sharp & Dohme Corp./COG	Shahab Asgharzadeh, MD	Magnolia Manzano mmanzano@chla.usc.edu
NCT02677116 <i>Phase I</i>	15B-MC-JGDN A Phase I, 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	Lilly	Leo Mascarenhas, MD	Magnolia Manzano mmanzano@chla.usc.edu
NCT02298348 <i>Phase I</i>	NANT N2013-02 A Phase I Study of Sorafenib and Cyclophosphamide/Topotecan in Patients with Relapsed and Refractory Neuroblastoma	NANT	Araz Marachelian, MD	Beth Hasenauer chasenauer@chla.usc.edu
NCT01711554 <i>Phase I</i>	NANT 2011-04 A Phase I Study of Lenalidomide and Anti-GD2 Mab Ch14.18 +/- Isotretinoin in Patients with Refractory/Recurrent Neuroblastoma	NANT	Araz Marachelian, MD	Beth Hasenauer chasenauer@chla.usc.edu
NCT02030964 <i>Phase I</i>	NANT 2012-01 A Phase I Study of Difluoromethylornithine (DMFO) and Celecoxib with Cyclophosphamide/Topotecan for Patients with Relapsed or Refractory Neuroblastoma	NANT	Araz Marachelian, MD	Beth Hasenauer chasenauer@chla.usc.edu
NCT02094625 <i>Phase I</i>	NAC A Dose-Finding Study of N-Acetylcysteine(NAC, IND122400) to Prevent Cisplatin-induced Hearing Loss in Children with Cancer	CHLA	Etan Orgel, MD	Marysol Gonzalez marygonzalez@chla.usc.edu
NCT02793466 <i>Phase I</i>	A Phase I, Open-Label, Single Institution Study to Assess the Safety, Tolerability, and Pharmacokinetics of Durvalumab in Pediatric Patients with Relapsed or Refractory Solid Tumors, Lymphoma, and Central Nervous System Tumors	CHLA	Leo Mascarenhas, MD	Hemlata Sukhija hsukhija@chla.usc.edu

NCT02601937 <i>Phase I</i>	EZH-102 A Phase I Study of the EZH2 Inhibitor Tazemetostat in Pediatric Subjects with Relapsed or Refractory INI1-Negative Tumors or Synovial Sarcoma	Epizyme	Ashley Margol, MD	Jasmine Pauly jpaul@chla.usc.edu
NCT02800889 <i>Phase I</i>	A Phase I, Dose-Escalation Study of Pixantrone Monotherapy in Pediatric Patients with Relapsed or Refractory Cancer	CTI Bio Pharma	Leo Mascarenhas, MD	Magnolia Manzano mmanzano@chla.usc.edu
NCT02780804 <i>Phase I</i>	ADVL1513 A Phase I Study of Entinostat , and Oral Histone Deacetylase Inhibitor, in Pediatric Patients with Recurrent or Refractory Solid Tumors, including CNS tumors and Lymphoma	COG	Fariba Navid, MD	Hemlata Sukhija hsukhija@chla.usc.edu
NCT02997189 <i>Phase II</i>	OTO-104 A multicenter, Phase 2 study to assess the feasibility, safety and efficacy of OTO-104 given by intratympanic administration in subjects at risk from cisplatin chemotherapy regimens in the treatment of cancer	Otonomy, Inc.	David Freyer, DO, MS	Marysol Gonzalez marygonzalez@chla.usc.edu
NCT02808650 <i>Phase I</i>	ADVL1515 A Phase I Study of Prexasertib , a CHK1/2 Inhibitor, in Pediatric Patients w/ Recurrent or Refractory Solid Tumors, including CNS Tumors	COG	Fariba Navid, MD	Hemlata Sukhija hsukhija@chla.usc.edu

Leukemia/Lymphoma				
NCT Number	Title	Sponsor	Investigator	Contact
NCT02625480 <i>Phase I/II</i>	KTE C19 A Phase I/II Multi-Center Study Evaluating the Safety and Efficacy of KTE C19 in Pediatric and Adolescent Subjects with Relapsed/ Refractory B-precursor Acute Lymphoblastic Leukemia (ZUMA-4)	Kite Pharma	Alan Wayne, MD	Renna Killen rkillen@chla.usc.edu
NCT02793466 <i>Phase I</i>	A Phase I, Open-Label, Single Institution Study to Assess the Safety, Tolerability, and Pharmacokinetics of Durvalumab in Pediatric Patients with Relapsed or Refractory Solid Tumors, Lymphoma, and Central Nervous System Tumors	CHLA	Leo Mascarenhas, MD	Hemlata Sukhija hsukhija@chla.usc.edu
NCT02538965 <i>Phase II</i>	AAML1522 A Phase II, Multicenter, Single-arm, Open-label Study to Evaluate the Activity, Safety and Pharmacokinetics of Lenalidomide (Revlimid®) in Pediatric Subjects From 1 to = 18 Years of Age With Relapsed/Refractory Acute Myeloid Leukemia	COG/Celgene	Deepa Bhojwani, MD	Stephanie Garcia stegarcia@chla.usc.edu
NCT01614197 <i>Phase I</i>	T2014-001 A Phase I Trial of Temsirolimus (CCI-779, Pfizer, Inc.) in Combination with Etoposide and Cyclophosphamide in Children with Relapsed Acute Lymphoblastic Leukemia and Non-Hodgkins Lymphoma	TACL	Paul Gaynon, MD	Nisha Bhagat nbhagat@chla.usc.edu
NCT02879643 <i>Phase I</i>	T2012-002 TACL Vincristine Sulfate Liposome Injection (Marqibo®) in Combination with UK ALL R3 Induction Chemotherapy for Children, Adolescents, and Young Adults with Relapsed ALL	TACL/ Spectrum	Alan Wayne, MD	Nisha Bhagat nbhagat@chla.usc.edu

NCT02800889 <i>Phase I</i>	A Phase I, Dose-Escalation Study of Pixantrone Monotherapy in Pediatric Patients with Relapsed or Refractory Cancer	CTI Bio Pharma	Leo Mascarenhas, MD	Magnolia Manzano mmanzano@chla.usc.edu
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Neuro-oncology				
NCT Number	Title	Sponsor	Investigator	Contact
NCT02677116 <i>Phase I</i>	A Phase I, 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	Lilly	Leo Mascarenhas, MD	Magnolia Manzano mmanzano@chla.usc.edu
NCT02285439 <i>Phase I/II</i>	MEK162 Phase I/II Study of MEK162 for Children With Low-Grade Gliomas and Other Ras/Raf/ERK Pathway Activated Tumors	CHLA	Nathan Robison, MD	Jasmine Pauly jpauly@chla.usc.edu
NCT02793466 <i>Phase I</i>	A Phase I, Open-Label, Single Institution Study to Assess the Safety, Tolerability, and Pharmacokinetics of Durvalumab in Pediatric Patients with Relapsed or Refractory Solid Tumors, Lymphoma, and Central Nervous System Tumors	CHLA	Leo Mascarenhas, MD	Hemlata Sukhija hsukhija@chla.usc.edu
NCT02094625 <i>Phase I</i>	NAC A Dose-Finding Study of N Acetylcysteine (NAC, IND122400) to Prevent Cisplatin-induced Hearing Loss in Children with Cancer	CHLA	Etan Orgel, MD	Hemlata Sukhija hsukhija@chla.usc.edu
NCT02717455 <i>Phase I</i>	PBTC-047: Phase I Trial of Panobinostat in Children with Diffuse Intrinsic Pontine Glioma, v2.0	PBTC	Girish Dhall, MD	Hugo Yepez huyepez@chla.usc.edu
NCT01089101 <i>Phase I/II</i>	PBTC 029B Phase I/II and Re-Treatment Study of AZD6244 for Recurrent or Refractory Pediatric Low Grade Glioma	PBTC	Girish Dhall, MD	Hugo Yepez huyepez@chla.usc.edu
NCT02601937 <i>Phase I</i>	EZH-102 A Phase I Study of the EZH2 Inhibitor Tazemetostat in Pediatric Subjects with Relapsed or Refractory INI1-Negative Tumors or Synovial Sarcoma	Epizyme	Ashley Margol, MD	Jasmine Pauly jpauly@chla.usc.edu
NCT02255461 <i>Phase I</i>	PBTC-042 A Phase I Study of CDK 4-6 Inhibitor PD-0332991 in Children with Recurrent, Progressive or Refractory CNS Tumors	PBTC	Girish Dhall, MD	Hugo Yepez huyepez@chla.usc.edu
NCT01748149 <i>Phase 0</i>	PNOC-002 A Safety, Phase 0, Pilot Efficacy Study of Vemurafenib , an Oral Inhibitor of BRAFV600E, in Children with Mutant Gliomas	PNOC/ Genentech, Inc.	Ashley Margol, MD	Patricia Diaz padiaz@chla.usc.edu
NCT01964300 <i>Phase II</i>	PBTC 039 Phase II Study of Peginterferon alfa-2b for Pediatric Patients with Unresectable or Recurrent Craniopharyngioma	PBTC	Girish Dhall, MD	Hugo Yepez huyepez@chla.usc.edu
NCT02359565 <i>Phase II</i>	PBTC 045 A Safety and Preliminary Efficacy Trial of MK-3475 (Pembrolizumab, anti-PD-1) in Children with Recurrent, Progressive or Refractory High-Grade Gliomas (HGG) and DIPGs	PBTC	Girish Dhall, MD	Hugo Yepez huyepez@chla.usc.edu
NCT01326104 <i>Phase II</i>	Re-MATCH Recurrent Medulloblastoma and Primitive Neuroectodermal Tumor Adoptive T Cell Therapy During Recovery From Myeloablative Chemotherapy and Hematopoietic Stem Cell Transplantation	USF	Girish Dhall, MD	Jasmine Pauly jpauly@chla.usc.edu

NCT01767792 <i>Phase II</i>	AVF4807 Open-label, Phase II Study of Bevacizumab in Children and Young Adults with Neurofibromatosis 2 and Progressive Vestibular Schwannomas that are Poor Candidates for Standard Treatment with Surgery or Radiation	NF Consortium/ Genentech, Inc.	Girish Dhall, MD	Patricia Diaz padiaz@chla.usc.edu
NCT02637687 <i>Phase I</i>	A Phase I Study of the Oral TRK Inhibitor LOXO-101 in Pediatric Patients With Advanced Solid or Primary Central Nervous System Tumors	LOXO Oncology	Leo Mascarenhas, MD	Hemlata Sukhija hsukhija@chla.usc.edu

HEMATOLOGY

NCT Number	Title	Sponsor	Investigator	Contact
NCT02582060 <i>Pilot</i>	HIP TEG Individualizing Hemophilia Prophylaxis Using Thromboelastography	CHLA	Guy Young, MD	Jeanie Lee hslee@chla.usc.edu
NCT02564718 <i>Phase I/II</i>	Bay 17618 A 7-day Study of the Safety, Efficacy and the Pharmacokinetic and Pharmacodynamic Properties of Oral Rivaroxaban in Children from Birth to Less than 6 Months with Catheter-related Arterial or Venous Thrombosis	Bayer/ Janssen	Guy Young, MD	Amy Stillings astillings@chla.usc.edu
NCT02418793 <i>Phase I/II</i>	MOD-5014 Phase I/II, Open-label, multicenter, Dose Escalation Study to Assess the Safety and Pharmacokinetics of Long Acting Recombinant FVIIa in Adult Men with Hemophilia A or B	OPKO Biologics, Ltd.	Guy Young, MD	Roxana Carmona rcarmona@chla.usc.edu

BLOOD AND MARROW TRANSPLANTATION

NCT Number	Title	Sponsor	Investigator	Contact
NCT02646839 <i>Phase I/II</i>	KIR FAVORABLE The Role of KIR-favorably Mismatched Haploidentical Transplantation and KIR-polymorphisms in Determining Outcomes of Children with ALL/AML/MDS Undergoing Allogeneic Hematopoietic Cell Transplantation	CHLA	Michael Pulsipher, MD	Greg Balagot gbalagot@chla.usc.edu
NCT01743131 <i>Phase II</i>	Abatacept GVH1201 Combined with a Calcineurin Inhibitor and Methotrexate for Graft Versus Host Disease Prophylaxis	CHLA	Michael Pulsipher, MD	Hugo Yopez huyopez@chla.usc.edu
NCT02065869 <i>Phase I/II</i>	BP-004 Phase I/II Study of CaspaCide T cells from an HLA-partially Matched Family Donor after Negative Selection of TCR $\alpha\beta$ +T Cells in Pediatric Patients Affected by Hematological Disorders	Bellicum	Neena Kapoor, MD	Renna Killen rkillen@chla.usc.edu
NCT02452034 <i>Phase I</i>	MK-5592 A Study of the Safety, Tolerability, and Pharmacokinetics of Intravenous (IV) and Powder for Oral Suspension Formulations of Posaconazole (POS) in Immunocompromised Pediatric Subjects with Neutropenia	Merck	Hisham Abdel-Azim, MD	Greg Balagot gbalagot@chla.usc.edu

NCT02028455 Phase I/II	PLAT-02 Pediatric and Young Adult Leukemia Adoptive Therapy: A Phase I/II Feasibility and Safety Study of CD19-CAR T Cell Immunotherapy for CD19+ Leukemia	Seattle Children's Hospital	Michael Pulsipher, MD	Brittany Toda btoda@chla.usc.edu
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NCI - National Cancer Institute

NANT – New Approaches to Neuroblastoma Therapy Consortium

TACL – Therapeutic Advances in Childhood Leukemia Consortium

NF Consortium – Neurofibromatosis Clinical Trials Consortium

USF – University of South Florida

COG – Children's Oncology Group

SARC – Sarcoma Alliance for Research through Collaboration

PBTC – Pediatric Brain Tumor Consortium

CHLA – Children's Hospital Los Angeles

PNOG – Pacific Pediatric Neuro-Oncology Consortium

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