Lucile Packard Children's Hospital Stanford

Bass Center for Childhood Cancer and Blood Diseases

Phase I and Phase II Clinical Trials: Hematology, Oncology, and Stem Cell Transplantation

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Oncology: Leukemia/Lymphoma

NCT #/Phase	Title	Sponsor	Investigator	Contact
Phase II/III (NCT03117751)	**TOT17: Total Therapy XVII for Newly Diagnosed Patients With Acute Lymphoblastic Leukemia and Lymphoma**	St. Jude	Norman Lacayo	Regina Dagher, NP, rdagher@stanford.edu
Phase II (NCT03164057)	**AML16: Phase II Trial of Epigenetic Priming in Patients with Newly Diagnosed Acute Myeloid Leukemia**	St. Jude	Norman Lacayo	Stefania Chirita, schirita@stanford.edu
Phase I (NCT02553460)	**TINI: Total Therapy for Infants with Acute Lymphoblastic Leukemia (ALL)**	St. Jude	Norman Lacayo	Stefania Chirita, schirita@stanford.edu
Phase II (NCT03755804)	**Pediatric Classical Hodgkin Lymphoma Consortium Study: cHOD17**	St. Jude	Michael Link	Stefania Chirita, schirita@stanford.edu
Phase I (NCT02512926)	**POE 14-01: Study of Carfilzomib in combination with Cyclophosphamide and Etoposide for Children with Relapsed or Refractory Solid Tumors and Leukemias**	POETIC	Norman Lacayo	Amy Li, ali4@stanford.edu
Phase I/II (NCT02932280)	**POE 16-01: A Phase I/II study of Neratinib in Pediatric Patients with Relapse/Refractory Solid Tumors or Hematologic Malignancies**	POETIC	Norman Lacayo	Amy Li, ali4@stanford.edu
Phase II (NCT01979536)	**ANHL12P1: A Randomized Phase II Trial of Brentuximab Vedotin or Crizotinib in Combination with Chemotherapy for Newly Diagnosed Patients with Anaplastic Large Cell Lymphoma (ALCL)**	COG	Jay Balagtas	Nancy Sweeters, RN, nancy.sweeters@stanford.edu
Phase I (NCT03792256)	**A Phase 1 Study of Palbociclib (IND#141416), a CDK 4/6 Inhibitor, in Combination with Chemotherapy in Children with Relapsed Acute Lymphoblastic Leukemia (ALL) or Lymphoblastic Lymphoma (LL)**	COG	Jay Balagtas	Stefania Chirita, schirita@stanford.edu

NCT #/Phase	Title	Sponsor	Investigator	Contact
Phase I/II (coming soon)	**IDASA: A Phase I/II, Multicenter, Open-Label, Multi-Arm Study Evaluating the Safety, Tolerability, Pharmacokinetics, and Preliminary Activity of Idasanutlin In Combination with Either Chemotherapy or Venetoclax in the Treatment of Pediatric and Young Adult Patients with Relapsed/Refractory Acute Leukemias or Solid Tumors**	Genentech	Norman Lacayo	Amy Li, ali4@stanford.edu

Oncology: Immunotherapy

NCT #/Phase	Title	Sponsor	Investigator	Contact
Phase I (NCT04196413)	**Phase I Clinical Trial of Autologous GD2 Chimeric Antigen Receptor (CAR) T cells (GD2CART) for Diffuse Intrinsic Pontine Gliomas (DIPG) and Spinal Diffuse Midline Glioma (DMG)**	Stanford	Crystal Mackall and Michelle Monje	Christina Baggott, PhD, baggott@stanford.edu
Phase IB (NCT03448393)	**Phase IB Clinical Trial of Autologous CD22 Chimeric Antigen Receptor (CAR) T Cells in Children and Young Adults With Recurrent or Refractory B Cell Malignancies (GENE TRANSFER)**	Stanford	Liora Schultz	Christina Baggott, PhD, baggott@stanford.edu
Phase I	**Phase 1 Dose Escalation Study of CD19/CD22 Chimeric Antigen Receptor (CAR) T Cells in Children and Young Adults with Recurrent or Refractory B Cell Malignancies**	Stanford	Crystal Mackall	Christina Baggott, PhD, baggott@stanford.edu
Phase II (NCT02900976)	**ANHL1522: A Pilot Study of Rituximab (RTX) and Third Party Latent Membrane Protein (LMP)-specific Cytotoxic T-Lymphocytes (LMP-TC, IND # 17068) in Pediatric Solid Organ Recipients (SOT) with EBV-Positive CD20-Positive Post-Transplant Lymphoproliferative Disease (PTLD)**	COG	Jay Balagtas	Hina Raheel, hraheel@stanford.edu
Phase II (NCT03876769)	**AALL1721/CASSIOPEIA: A phase II trial of tisagenlecleucel in first-line high-risk (HR) pediatric and young adult patients with B-cell acute lymphoblastic leukemia (B-ALL) who are minimal residual disease (MRD) positive at the end of consolidation (EOC) therapy**	Novartis/ COG	Kara Davis	Christina Baggott, PhD, baggott@stanford.edu
Phase I (NCT03904069, coming soon)	**CCT5027: A Phase 1 Study Evaluating the Safety, Tolerability, and Efficacy of FLT3 Chimeric Antigen Receptor T-cell (CAR-T) AMG 553 in Subjects With Relapsed/Refractory Acute Myeloid Leukemia**	Janssen	Lori Muffly, Liora Schultz	Christina Baggott, PhD, baggott@stanford.edu
Phase II (NCT03384654)	**An Open-label, Multicenter, Phase 2 Study Evaluating the Efficacy and Safety of Daratumumab in Pediatric and Young Adult Subjects ≥1 and ≤30 Years of Age With Relapsed/Refractory Precursor B-cell or T-cell Acute Lymphoblastic Leukemia or Lymphoblastic Lymphoma**	Janssen	Liora Schultz	Christina Baggott, PhD, baggott@stanford.edu

Oncology: Neuro-Oncology

NCT #/Phase	Title	Sponsor	Investigator	Contact
Phase II/III (NCT02114229)	**SJATRT: Phase 2 Study Of Alisertib as a Single Agent in Recurrent or Progressive Central Nervous System (CNS) Aypicial Teratoid Rhabdoid Tumors (AT/RTs) and Extra-CNS Malignant Rhabdoid Tumors (MRTs) and in Combination Therapy in Newly Diagnosed AT/RT**	St. Jude	Sonia Partap	Stefania Chirita, schirita@stanford.edu
Phase II/III (NCT01878617)	**SJMB12: A Clinical and Molecular Risk-Directed Therapy for Newly Diagnosed Medulloblastoma**	St. Jude	Sonia Partap	Stefania Chirita, schirita@stanford.edu
Phase II (NCT02867592)	**ADVL1622: Phase 2 Trial of XL184 (Cabozantinib) an Oral Small-Molecule Inhibitor of Multiple Kinases, in Children and Young Adults with Refractory Sarcomas, Wilms Tumor and Other Rare Tumors**	COG	Jay Balagtas	Erica Velasco, ericav@stanford.edu
Phase II (NCT03581292)	**ACNS1721: A Phase 2 Study of Veliparib (ABT-888, IND # 139199) and Local Irradiation, Followed by Maintenance Veliparib and Temozolomide, in Patients with Newly Diagnosed High-Grade Glioma (HGG) without H3 K27M or BRAFV600E Mutations COG**	COG	Jay Balagtas	Stefania Chirita, schirita@stanford.edu
Phase I (coming soon)	**PBTC-56: A phase I study of the ADAM-10 inhibitor INCB7839 in children with recurrent/progressive high-grade gliomas to target microenvironmental neuroligin-3**	PBTC	Michelle Monje	Erica Velasco, ericav@stanford.edu
Phase I (NCT03389802)	**PBTC-051: Phase I Study to Evaluate the Safety and Tolerability of the CD40 Agonistic Monoclonal Antibody APX005M in Pediatric Subjects With Recurrent/ Refractory Brain Tumors and Newly Diagnosed Brain Stem Glioma**	PBTC	Michelle Monje	Stefania Chirita, schirita@stanford.edu
Phase I (NCT03598244)	**PBTC-49: A Phase I study of Savolitinib in Recurrent, Progressive or Refractory Medulloblastoma, High-Grade Glioma, or Diffuse Intrinsic Pontine Glioma**	PBTC	Michelle Monje	Erica Velasco, ericav@stanford.edu
Feasibility Study (NCT03033992)	**PBTC-48: Feasibility trial of Optune for children with recurrent or progressive supratentorial high-grade glioma and ependymoma**	PBTC	Michelle Monje	Stefania Chirita, schirita@stanford.edu
Phase I/II (NCT02717455)	**PBTC-047: Phase 1 Trial of Panobinostat in Children with Diffuse Intrinsic Pontine Glioma**	PBTC	Michelle Monje	Erica Velasco, ericav@stanford.edu
Phase I/II (NCT02359565)	**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), DIPGs and hypermutated brain tumors**	PBTC	Michelle Monje	Stefania Chirita, schirita@stanford.edu

NCT #/Phase	Title	Sponsor	Investigator	Contact
Phase I/II (NCT01089101)	**PBTC-029B: A Phase I and Phase II and Re-Treatment Study of AZD6244 for Recurrent or Refractory Pediatric Low Grade Glioma**	PBTC	Michelle Monje	Erica Velasco, ericav@stanford.edu
Phase IIb (coming soon)	**A Phase 2b Trial of the MEK 1/2 Inhibitor (MEKi) PD-0325901 in Adult and Pediatric Patients with Neurofibromatosis Type 1 (NF1)-Associated Inoperable Plexiform Neurofibromas (PNs) that are Progressing or Causing Significant Morbidity**	Spring-Works	Cynthia Campen	Erica Velasco, ericav@stanford.edu

Oncology: Solid Tumors

NCT #/Phase	Title	Sponsor	Investigator	Contact
Phase I/II (NCT02932280)	**POE 16-01: A Phase I/II study of Neratinib in Pediatric Patients with Relapse/Refractory Solid Tumors or Hematologic Malignancies**	POETIC	Norman Lacayo	Amy Li, ali4@stanford.edu
Phase I (NCT02512926)	**POE 14-01: Phase I Study of Carfilzomib in combination with Cyclophosphamide and Etoposide for Children with Relapsed or Refractory Solid Tumors and Leukemias**	POETIC	Norman Lacayo	Amy Li, ali4@stanford.edu
Phase I/II (coming soon)	**IDASA: A Phase I/II, Multicenter, Open-Label, Multi-Arm Study Evaluating the Safety, Tolerability, Pharmacokinetics, and Preliminary Activity of Idasanutlin In Combination with Either Chemotherapy or Venetoclax in the Treatment of Pediatric and Young Adult Patients with Relapsed/ Refractory Acute Leukemias or Solid Tumors**	Genentech	Norman Lacayo	Amy Li, ali4@stanford.edu
Phase I	**Cellectar: A Phase 1, Open-Label, Dose Escalation Study of CLR 131 in Children and Adolescents with Select Solid Tumors, Lymphoma, and Malignant Brain Tumors**	Cellectar	Norman Lacayo	Hina Raheel, hraheel@stanford.edu
Phase I/II	**A Phase 1/2 Study of the Oral RET Inhibitor LOXO-292 in Pediatric Patients with Advanced RET-Altered Solid or Primary Central Nervous System Tumors**	Loxo	Sheri Spunt	Hina Raheel, hraheel@stanford.edu
Phase II (coming soon)	**ADVL1921: Phase 1 study to evaluate the safety and pharmacokinetics of palbociclib (IBRANCE®) in combination with irinotecan and temozolomide in pediatric patients with recurrent or refractory solid tumors**	COG/ Pfizer	Jay Balagtas	Hina Raheel, hraheel@stanford.edu

Stem Cell Transplantation

NCT #/Phase	Title	Sponsor	Investigator	Contact
Phase II (NCT04249830)	**Allogeneic hematopoietic stem cell transplantation from an HLA-partially matched related or unrelated donor after TCR αβ+T cells/CD19+ B cell depletion in children and young adults affected by malignant or non-malignant hematological disorders.**	Stanford	Alice Bertaina	Nivedita Kunte, nkunte@stanford.edu
Expanded Access (NCT02162511)	**An Expanded Access Study Using the CliniMACS System to Offer Therapeutic Manipulated Grafts that are CD34 Cell Enriched and T Cell Depleted for Allogeneic Stem Cell Recipients (BMT 271)**	Stanford	Rajni Agarwal-Hashmi	Nivedita Kunte, nkunte@stanford.edu
Phase I (NCT02963064)	**A Study to Evaluate the Safety and Tolerability of Tandemly Purified Allogeneic CD34+ CD90+ Hematopoietic Stem Cells (HSC) Administered Following Conditioning with AMG 191 to Achieve Engraftment and Immune Reconstitution in Patients with Severe Combined Immunodeficiency (SCID)**	Jasper Thera-peutics	Rajni Agarwal-Hashmi	Elisabeth Merkel, RN, merkel@stanford.edu
Phase I/II (NCT03198234)	**Use of T-allo10 cell infusions combined with mismatched related or unrelated donor hematopoietic stem cell transplantation (HSCT) for hematological malignancies**	Stanford	Rajni Agarwal-Hashmi	Nivedita Kunte, nkunte@stanford.edu
Pilot Study (NCT02845596)	**Unrelated Donor Transplant Versus Immune Therapy in Pediatric Severe Aplastic Anemia (TransIT)**	Pediatric Blood and Marrow Transplant Consortium	Alice Bertaina	Alicia Harnett, aharnett@stanford.edu
Phase 2 (NCT03619551)	**A randomized trial of low versus moderate exposure busulfan for infants with severe combined immunodeficiency (SCID) receiving TCRab+/CD19+ depleted transplantation: A Phase II Study (CSIDE)**	Pediatric Blood and Marrow Transplant Consortium	Alice Bertaina	Alicia Harnett, aharnett@stanford.edu
Phase II (NCT02646839)	**KIR Favorable Mismatched Haplo Transplant and KIR Polymorphism in ALL/AML/MDS Allo-HCT Children**	Pediatric Blood and Marrow Transplant Consortium	Alice Bertaina	Nivedita Kunte, nkunte@stanford.edu
Phase I (NCT04640987)	**A Study of T-allo10 Infusion After HLA-Partially Matched Related or Unrelated TCR αβ+ T-cell/ CD19+ B-cell Depleted Allogeneic Hematopoietic Stem Cell Transplantation (αβ Depleted-HSCT) in Children and Young Adults Affected by Hematologic Malignancies**	Stanford	Alice Bertaina	Nivedita Kunte, nkunte@stanford.edu

NCT #/Phase	Title	Sponsor	Investigator	Contact
Phase I (NCT04105166)	**Gene Therapy for Pyruvate Kinase Deficiency (PKD): A Phase I Clinical Trial to Evaluate the Safety of the Infusion of Autologous CD34 Cells Transduced with a Lentiviral Vector Carrying the Codon Optimized Red Cell Pyruvate Kinase (coRPK) Gene in Adult and Pediatric Subjects with PKD. (GENE TRANSFER)**	Rocket Pharma-ceuticals	Ami Shah	Elisabeth Merkel, RN, merkel@stanford.edu
Phase II (NCT04069533)	**A Phase II Clinical Trial to Evaluate the Safety of the Infusion of Autologous CD34+ Cells Transduced with a Lentiviral Vector Carrying the FANCA Gene in Pediatric Subjects with Fanconi Anemia Subtype-A**	Rocket Pharma-ceuticals	Agnieszka Czechowicz	Elisabeth Merkel, RN, merkel@stanford.edu
Phase III (NCT01896102)	**A Phase 3 Study of LentiD Drug Product After Myeloablative Conditioning Using Busulfan and Fludarabine in Subjects 17 Years of Age and Under With Cerebral Adrenoleukodystrophy (CALD) (GENE TRANSFER)**	bluebird bio	Ami Shah	Elisabeth Merkel, RN, merkel@stanford.edu
Phase I/II (NCT03745287)	**A Phase 1/2 Study to Evaluate the Safety and Efficacy of a Single Dose of Autologous CRISPRCas9 Modified CD34+ Human Hematopoietic Stem and Progenitor Cells (CTX001) in Subjects With Severe Sickle Cell Disease (GENE TRANSFER)**	Vertex	Ami Shah	Elisabeth Merkel, RN, merkel@stanford.edu
Phase I/II (NCT03655678)	**A Phase 1/2 Study of the Safety and Efficacy of a Single Dose of Autologous CRISPRCas9 Modified CD34+ Human Hematopoietic Stem and Progenitor Cells (hHSPCs) in subjects with Transfusion-Dependent Beta Thalassemia (GENE TRANSFER)**	Vertex	Ami Shah	Elisabeth Merkel, RN, merkel@stanford.edu

Hematology

NCT #/Phase	Title	Sponsor	Investigator	Contact
Phase 3 (NCT03587116 —coming soon)	**Gene Therapy: An Open-Label, Non-Investigational Product, Multi-Center, Lead-In Study to Evaluate at Least 6 Months of Prospective Efficacy and Selected Safety Data of Current Factor IX (FIX) or Factor VIII (FVIII) Prophylaxis Replacement Therapy in the Usual Care Setting of Moderately Severe to Severe Adult Hemophilia B subjects (FIX:C≤2%) Who Are Negative for Neutralizing Antibodies to Adeno-Associated Virus Vector-Spark100 (Benegene-1) and Moderately Severe to Severe Hemophilia A Adult Subjects (FVIII:C≤1%) Who Are Negative for Neutralizing Antibodies to Adeno-Associated Virus Vector SB-525 capsid (AAV6), Prior to the Respective Therapeutic Phase 3 Gene Therapy Studies**	Pfizer	May Chien	Liza Reichert, lreichert@stanford.edu
Phase 3 (NCT04370054 —coming soon)	**Gene Therapy: Open-label, Single-Arm Study to Evaluate the Efficacy and Safety of PF 07055480 (Recombinant AAV2/6 Human Factor VIII Gene Therapy) in Adult Male Participants with Moderately Severe to Severe Hemophilia A (FVIII:C≤1%)**	Pfizer	Caroline Berube	Liza Reichert, lreichert@stanford.edu