

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

## Contacts

General Inquiries: Oncology	Stefania Chirita Liza Reichert	<a href="mailto:schirita@stanford.edu">schirita@stanford.edu</a> <a href="mailto:lreichert@stanford.edu">lreichert@stanford.edu</a>
General Inquiries: Hematology	Liza Reichert	<a href="mailto:lreichert@stanford.edu">lreichert@stanford.edu</a>
General Inquiries: Stem Cell Transplantation	Rajni Agarwal, MD Alice Bertaina, MD, PhD Tami John, MD	<a href="mailto:rajnia@stanford.edu">rajnia@stanford.edu</a> <a href="mailto:Aliceb1@stanford.edu">Aliceb1@stanford.edu</a> <a href="mailto:tdjohn10@stanford.edu">tdjohn10@stanford.edu</a> <a href="mailto:DL-SCTIntakeCoordinators@stanfordchildrens.org">DL-SCTIntakeCoordinators@stanfordchildrens.org</a>
AML, ALL	Catherine Aftandilian, MD, MS Norman Lacayo, MD	<a href="mailto:aftandil@stanford.edu">aftandil@stanford.edu</a> <a href="mailto:lacayon@stanford.edu">lacayon@stanford.edu</a>
Bone and Soft Tissue Sarcomas, Renal Tumors, Liver Tumors, Germ Cell Tumors	Allison Pribnow, MD	<a href="mailto:apribnow@stanford.edu">apribnow@stanford.edu</a>
HLH	Michael Jeng, MD	<a href="mailto:mjeng@stanford.edu">mjeng@stanford.edu</a>
Hodgkin's and Non-Hodgkin's Lymphoma	Michael Link, MD Lianna Marks, MD	<a href="mailto:mink@stanford.edu">mink@stanford.edu</a> <a href="mailto:marksl@stanford.edu">marksl@stanford.edu</a>
Immunotherapy	Kara Davis, DO Crystal Mackall, MD Liora Schultz, MD	<a href="mailto:kardavis@stanford.edu">kardavis@stanford.edu</a> <a href="mailto:cmackall@stanford.edu">cmackall@stanford.edu</a> <a href="mailto:lioras@stanford.edu">lioras@stanford.edu</a>
Neuroblastoma, Retinoblastoma	Raya Saab, MD	<a href="mailto:rsaab@stanford.edu">rsaab@stanford.edu</a>

Neuro-Oncology	Cynthia Campen, MD Paul Fisher, MD Michelle Monje, MD Sonia Partap, MD	<a href="mailto:ccampen@stanford.edu">ccampen@stanford.edu</a> <a href="mailto:pfisher@stanford.edu">pfisher@stanford.edu</a> <a href="mailto:mmonje@stanford.edu">mmonje@stanford.edu</a> <a href="mailto:spartap@stanford.edu">spartap@stanford.edu</a>
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## Oncology: Leukemia/Lymphoma

NCT #/Phase	Title	Led by	Investigator	Contact
Phase I-II (NCT05848687)	<b>TINI 2: Total Therapy for Infants with Acute Lymphoblastic Leukemia II</b>	Stanford	Tanja Gruber	Tanja Gruber, MD, PhD, <a href="mailto:tagruber@stanford.edu">tagruber@stanford.edu</a>
Phase I (NCT05101551)	<b>PARPAML: A Phase 1 Protocol for Relapsed Pediatric AML to Determine the Safety and Efficacy of the PARP Inhibitor Talazoparib in Combination with Chemotherapy</b>	Stanford	Jennifer Kamens	Stefania Chirita, <a href="mailto:schirita@stanford.edu">schirita@stanford.edu</a>
Phase I (NCT04996160)	<b>Palbociclib in Combination With Chemotherapy in Pediatric Patients With Relapsed or Refractory Acute Lymphoblastic Leukemia (REL Pall2)</b>	Stanford	Tanja Gruber	Tanja Gruber, MD, PhD, <a href="mailto:tagruber@stanford.edu">tagruber@stanford.edu</a>
Phase I (NCT05188170)	<b>Phase 1 Study of Niclosamide in Pediatric and Young Adults Patients with Relapsed and Refractory AML</b>	Stanford	Kathleen Sakamoto	Stefania Chirita, <a href="mailto:schirita@stanford.edu">schirita@stanford.edu</a>
Phase II (NCT03755804)	<b>Pediatric Classical Hodgkin Lymphoma Consortium Study: cHOD17</b>	St. Jude	Michael Link	Stefania Chirita, <a href="mailto:schirita@stanford.edu">schirita@stanford.edu</a>
Phase II (NCT02981628)	<b>A Phase 2 Study of Inotuzumab Ozogamicin (NSC# 772518, IND# 133494) in Children and Young Adults with Relapsed or Refractory CD22+ B-Acute Lymphoblastic Leukemia (B-ALL)</b>	COG	Jay Balagtas	Stefania Chirita, <a href="mailto:schirita@stanford.edu">schirita@stanford.edu</a>
Phase I/II (NCT05255601)	<b>CA224069: A Phase 1/2 Study of the Safety, Tolerability, Pharmacokinetics and Preliminary Efficacy of Relatlimab Plus Nivolumab in Pediatric and Young Adult Participants with Recurrent or Refractory Classical Hodgkin Lymphoma and Non-Hodgkin Lymphoma</b>	Bristol-Myers Squibb	Lianna Marks	Stefania Chirita, <a href="mailto:schirita@stanford.edu">schirita@stanford.edu</a>
Phase I (NCT05206357)	<b>M20-429: A single arm, open-label, phase 1b trial of Epcoritamab in pediatric patients with relapsed/refractory aggressive mature B-cell neoplasms</b>	Genmab	Lianna Marks	Stefania Chirita, <a href="mailto:schirita@stanford.edu">schirita@stanford.edu</a>
Phase II (NCT04554914)	<b>A Study to Evaluate Tabelecleucel in Participants With Epstein-barr Virus-associated Diseases</b>	Atara Biotherapeutics	Lianna Marks	Chloe Ordonez, RN, <a href="mailto:cordona@stanford.edu">cordona@stanford.edu</a>

## Oncology: Immunotherapy

NCT #/Phase	Title	Led by	Investigator	Contact
Phase I (Coming soon)	<b>Phase 1 Clinical Trial Of Autologous B7-H3 Chimeric Antigen Receptor T Cells (B7-H3cart) In Children And Young Adults With Relapsed Or Refractory Solid Tumor Expressing B7-H3 Target</b>	Stanford	Sneha Ramakrishna	Amy Li, <a href="mailto:ali4@stanford.edu">ali4@stanford.edu</a>
Phase I/Ib NCT06408194	<b>Phase I/Ib Clinical Trial of Autologous CD22 Chimeric Antigen Receptor (CAR) T cells following Commercial CD19 CAR T cells in Children and Young Adults with Recurrent or Refractory B Cell Malignancies</b>	Stanford	Kara Davis	Michelle Fujimoto, <a href="mailto:mfujimot@stanford.edu">mfujimot@stanford.edu</a>
Phase I (NCT04196413)	<b>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)</b>	Stanford	Crystal Mackall and Michelle Monje	Christina Baggott, PhD, <a href="mailto:baggott@stanford.edu">baggott@stanford.edu</a>
Phase I (NCT03241940)	<b>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</b>	Stanford	Crystal Mackall	Christina Baggott, PhD, <a href="mailto:baggott@stanford.edu">baggott@stanford.edu</a>
Phase II (NCT05460533)	<b>Phase II Open Label Multicenter Study of Early REinFusion of Tisagenlecleucel to Promote Durable B-Cell Aplasia in Pediatric and Young Adult Patients with Relapsed/Refractory CD19-Positive B-Cell Acute Lymphoblastic Leukemia (REFUEL)</b>	Memorial Sloan Kettering	Liora Schultz	Sneha Jariwala, <a href="mailto:snehaj@stanford.edu">snehaj@stanford.edu</a>
Phase I/II (NCT05642455)	<b>A Phase 1/2, Open-Label, Basket Study to Assess the Safety, Tolerability, and Anti-Tumor Activity of Afamitresgene Autoleucel in Pediatric Subjects with MAGE-A4 Positive Tumors</b>	Adapt-immune	Sneha Ramakrishna	Alexandria Lim, <a href="mailto:alexlim@stanford.edu">alexlim@stanford.edu</a>
Phase I (NCT04539366)	<b>GD2-CAR PERSIST: Production and Engineering of GD2-Targeted, Receptor Modified T Cells (GD2CART) for Osteosarcoma or Neuroblastoma to Increase Systemic Tumor Exposure</b>	PED-CITN	Jay Balagtas	Sneha Jariwala, <a href="mailto:snehaj@stanford.edu">snehaj@stanford.edu</a>

## Oncology: Neuro-Oncology

NCT #/Phase	Title	Led by	Investigator	Contact
Phase I/II (NCT05286801)	<b>PEPN2121: A Phase 1/2 Study of Tiragolumab (NSC# 827799, IND# 161266) and Atezolizumab (NSC# 783608, IND# 161266) in Patients with Relapsed or Refractory SMARCB1 or SMARCA4 Deficient Tumors</b>	NCI	Lianna Marks	Stefania Chirita, <a href="mailto:schirita@stanford.edu">schirita@stanford.edu</a>
Phase I/II (NCT05099003)	<b>ACNS1821: A Phase 1/2 Trial of Selinexor (KPT-330) and Radiation Therapy in Newly-Diagnosed Pediatric Diffuse Intrinsic Pontine Glioma (DIPG) and High-Grade Glioma (HGG)</b>	National Cancer Institute	Jay Balagtas	Elisabeth Merkel, RN, <a href="mailto:merkel@stanford.edu">merkel@stanford.edu</a>
Phase II (NCT04684368)	<b>ACNS2021: Phase 2 Trial of Chemotherapy followed by Response-Based Whole Ventricular &amp; Spinal Canal Irradiation (WVSCI) for Patients with Localized Non-Germinomatous Central Nervous System Germ Cell Tumor</b>	COG	Jay Balagtas	Elisabeth Merkel, RN, <a href="mailto:merkel@stanford.edu">merkel@stanford.edu</a>
Phase II (NCT03919071)	<b>ACNS1723: Phase 2 Study of Dabrafenib (NSC# 763760) with Trametinib (NSC# 763093) after Local Irradiation in Newly-Diagnosed BRAFV600-Mutant High-Grade Glioma (HGG) (IND# 145355)</b>	COG	Jay Balagtas	Elisabeth Merkel, RN, <a href="mailto:merkel@stanford.edu">merkel@stanford.edu</a>
Phase II (NCT02724579)	<b>A Phase 2 Study of Reduced Therapy for Newly Diagnosed Average-Risk WNT-Driven Medulloblastoma (ACNS1422)</b>	COG	Jay Balagtas	Elisabeth Merkel, RN, <a href="mailto:merkel@stanford.edu">merkel@stanford.edu</a>
Phase I (NCT04978727)	<b>PBTC-060: A Pilot Study of SurVaxM in Children Progressive or Relapsed Medulloblastoma, High Grade Glioma, Ependymoma and Newly Diagnosed Diffuse Intrinsic Pontine Glioma</b>	PBTC	Sonia Partap	Elisabeth Merkel, RN, <a href="mailto:merkel@stanford.edu">merkel@stanford.edu</a>
Phase I/II (NCT04201457)	<b>PBTC-055: Phase I/II trial of Dabrafenib, Trametinib, and Hydroxychloroquine (HCQ) for BRAF V600E-mutant or Trametinib and HCQ for BRAF fusion/duplication positive or NF1-associated recurrent or progressive gliomas in children and young adults</b>	PBTC	Sonia Partap	Elisabeth Merkel, RN, <a href="mailto:merkel@stanford.edu">merkel@stanford.edu</a>
Phase I/II (NCT03904862)	<b>PBTC-053: A Pediatric Brain Tumor Consortium Phase I/ II and Surgical Study of CX-4945 in Patients with Recurrent SHH Medulloblastoma</b>	PBTC	Sonia Partap	Stefania Chirita, <a href="mailto:schirita@stanford.edu">schirita@stanford.edu</a>
Phase I (NCT03598244)	<b>PBTC-049: A Phase I study of Savolitinib in Recurrent, Progressive or Refractory Medulloblastoma, High-Grade Glioma, or Diffuse Intrinsic Pontine Glioma</b>	PBTC	Sonia Partap	Elisabeth Merkel, RN, <a href="mailto:merkel@stanford.edu">merkel@stanford.edu</a>

NCT #/Phase	Title	Led by	Investigator	Contact
Feasibility Study (NCT03033992)	<b>PBTC-048: Feasibility trial of Optune for children with recurrent or progressive supratentorial high-grade glioma and ependymoma</b>	PBTC	Sonia Partap	Stefania Chirita, <a href="mailto:schirita@stanford.edu">schirita@stanford.edu</a>
Phase I/II (NCT02359565)	<b>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</b>	PBTC	Sonia Partap	Stefania Chirita, <a href="mailto:schirita@stanford.edu">schirita@stanford.edu</a>
Phase I/II (NCT04774718)	<b>GO42286: A Phase I/II, Open-Label, Multicenter Study Evaluating the Safety, Pharmacokinetics, and Efficacy of Alectinib in Pediatric Patients w/ ALK-Fusion-Positive Solid or CNS Tumors for whom Prior Treatment has Proven to be Ineffective or for who there is no satisfactory treatment available</b>	Genentech	Sonia Partap	Elisabeth Merkel, RN, <a href="mailto:merkel@stanford.edu">merkel@stanford.edu</a>

## Oncology: Solid Tumors

NCT #/Phase	Title	Led by	Investigator	Contact
Phase II (NCT04616560)	<b>PEPN1924: A Phase 2 Study of DS-8201A (NSC# 807708, IND# 153036) in Adolescents, or Young Adults with Recurrent HER2+ Osteosarcoma</b>	NCI	Lianna Marks	Stefania Chirita, <a href="mailto:schirita@stanford.edu">schirita@stanford.edu</a>
Phase I/II (NCT05286801)	<b>PEPN2121: A Phase 1/2 Study of Tiragolumab (NSC# 827799, IND# 161266) and Atezolizumab (NSC# 783608, IND# 161266) in Patients with Relapsed or Refractory SMARCB1 or SMARCA4 Deficient Tumors</b>	National Cancer Institute	Lianna Marks	Stefania Chirita, <a href="mailto:schirita@stanford.edu">schirita@stanford.edu</a>
Phase II (NCT037943490)	<b>A Phase 2 Randomized Study of Irinotecan/ Temozolomide/Dinutuximab with or without Eflornithine (DFMO) (IND# 141913) in Children with Relapsed, Refractory or Progressive Neuroblastoma (ANBL1821)</b>	COG	Jay Balagtas	Chloe Ordon, RN, <a href="mailto:cordona@stanford.edu">cordona@stanford.edu</a>
Phase II (NCT04322318)	<b>AREN1921: A Study of Combination Chemotherapy for Patients With Newly Diagnosed DAWT and Relapsed FHW T</b>	COG	Jay Balagtas	Chloe Ordon, RN, <a href="mailto:cordona@stanford.edu">cordona@stanford.edu</a>
Phase I/II (NCT05286801)	<b>A Phase 1/2 Study of Tiragolumab (NSC# 827799, IND# 161266) and Atezolizumab (NSC# 783608, IND# 161266) in Patients with Relapsed or Refractory SMARCB1 or SMARCA4 Deficient Tumors</b>	COG	Jay Balagtas	Chloe Ordon, RN, <a href="mailto:cordona@stanford.edu">cordona@stanford.edu</a>
Phase I/II (NCT04901702)	<b>A Randomized Phase I/II study of Onivyde in combination with Talazoparib or Temozolomide in children and young adults with recurrent solid malignancies and Ewing sarcoma</b>	St. Jude	Allison Pribnow	Richard Fu, <a href="mailto:yifu@stanford.edu">yifu@stanford.edu</a>
Phase I/II (NCT05734066)	<b>A phase 1/2, open-label study to evaluate the safety, tolerability, pharmacokinetics (PK), recommended phase 2 dose (RP2D), and efficacy of lurbinectedin monotherapy in pediatric participants with previously treated solid tumors followed by expansion to assess efficacy and safety in pediatric and young adult participants with relapsed/refractory Ewing sarcoma</b>	Jazz Pharmaceuticals	Allison Pribnow	Richard Fu, <a href="mailto:yifu@stanford.edu">yifu@stanford.edu</a>
Phase I (NCT03478462)	<b>Collectar: A Phase 1, Open-Label, Dose Escalation Study of CLR 131 in Children and Adolescents with Select Solid Tumors, Lymphoma, and Malignant Brain Tumors</b>	Collectar	Allison Pribnow	Chloe Ordon, RN, <a href="mailto:cordona@stanford.edu">cordona@stanford.edu</a>
Phase II (NCT03709680)	<b>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</b>	COG/ Pfizer	Jay Balagtas	Chloe Ordon, RN, <a href="mailto:cordona@stanford.edu">cordona@stanford.edu</a>

## Stem Cell Transplantation and Gene Therapy

NCT #/Phase	Title	Led by	Investigator	Contact	Condition Treated
Phase I/II (NCT05508009)	<b>Phase 1b/2a trial of allogeneic hematopoietic stem cell transplantation (HSCT) from an HLA-partially matched related or unrelated donor after TCR<math>\alpha\beta</math> + T-cell/CD19+ B-cell depletion for patients who will receive a kidney transplant (KT) from the same HSCT donor</b>	Stanford	Alice Bertaina and Paul Grimm	<a href="mailto:DL-SCTIntakeCoordinators@stanfordchildrens.org">DL-SCTIntakeCoordinators@stanfordchildrens.org</a>	Non-malignant disorders
Phase I (NCT05241444)	<b>Phase I Study of Autologous CD4<sup>+</sup>LVFOXP3 in Participants With Immune Dysregulation Polyendocrinopathy Enteropathy X-linked (IPEX) Syndrome</b>	Stanford	Rajni Agarwal and Rosa Bacchetta	<a href="mailto:DL-SCTIntakeCoordinators@stanfordchildrens.org">DL-SCTIntakeCoordinators@stanfordchildrens.org</a>	Non-malignant disorders
Phase 1B/2A (NCT04784052)	<b>TCR<math>\alpha\beta</math>+T-CELL/CD19+B depleted Hematopoietic Grafts and A Reduced-Intensity Preparative Conditioning Regimen Containing Briquilimab to Achieve Engraftment and Blood Reconstitution in Patients with Fanconi Anemia</b>	Stanford	Rajni Agarwal	<a href="mailto:DL-SCTIntakeCoordinators@stanfordchildrens.org">DL-SCTIntakeCoordinators@stanfordchildrens.org</a>	Non-malignant disorders
Phase II (NCT04249830)	<b>Allogeneic hematopoietic stem cell transplantation from an HLA-partially matched related or unrelated donor after TCR <math>\alpha\beta</math>+T cells/CD19+ B cell depletion in children and young adults affected by malignant or non-malignant hematological disorders</b>	Stanford	Alice Bertaina	<a href="mailto:DL-SCTIntakeCoordinators@stanfordchildrens.org">DL-SCTIntakeCoordinators@stanfordchildrens.org</a>	Hematologic malignancies and non-malignant disorders
Phase I (NCT04640987)	<b>A Study of T-allo10 Infusion After HLA-Partially Matched Related or Unrelated TCR <math>\alpha\beta</math>+ T-cell/ CD19+ B-cell Depleted Allogeneic Hematopoietic Stem Cell Transplantation (<math>\alpha\beta</math> Depleted-HSCT) in Children and Young Adults Affected by Hematologic Malignancies</b>	Stanford	Alice Bertaina	<a href="mailto:DL-SCTIntakeCoordinators@stanfordchildrens.org">DL-SCTIntakeCoordinators@stanfordchildrens.org</a>	Hematologic malignancies
Phase I/II (NCT04819841)	<b>Gene Correction in Autologous CD34+ Hematopoietic Stem Cells (HbS to HbA) to Treat Severe Sickle Cell Disease (RESTORE)Study (CSIDE)</b>	Kamau Therapeutics	David Shyr	<a href="mailto:DL-SCTIntakeCoordinators@stanfordchildrens.org">DL-SCTIntakeCoordinators@stanfordchildrens.org</a>	Non-malignant disorders
Phase I/II (NCT05456880)	<b>BEACON: A Study Evaluating the Safety and Efficacy of BEAM-101 in Patients With Severe Sickle Cell Disease (BEACON)</b>	Beam Therapeutics	Tami John	<a href="mailto:DL-SCTIntakeCoordinators@stanfordchildrens.org">DL-SCTIntakeCoordinators@stanfordchildrens.org</a>	Non-malignant disorders

NCT #/Phase	Title	Led by	Investigator	Contact	Condition Treated
Phase I/II (NCT04522375)	<b>FuschiA Study-A Dose Escalation Study of FP-045 in Patients With Fanconi Anemia</b>	Foresee Therapeutics	Rajni Agarwal	<a href="mailto:DL-SCTIntakeCoordinators@stanfordchildrens.org">DL-SCTIntakeCoordinators@stanfordchildrens.org</a>	Non-malignant disorders
Phase 2 (NCT03619551)	<b>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)</b>	Pediatric Blood and Marrow Transplant Consortium	Alice Bertaina	<a href="mailto:DL-SCTIntakeCoordinators@stanfordchildrens.org">DL-SCTIntakeCoordinators@stanfordchildrens.org</a>	Non-malignant disorders
Phase II (NCT02646839)	<b>KIR Favorable Mismatched Haplo Transplant and KIR Polymorphism in ALL/AML/MDS Allo-HCT Children</b>	Pediatric Blood and Marrow Transplant Consortium	Alice Bertaina	<a href="mailto:DL-SCTIntakeCoordinators@stanfordchildrens.org">DL-SCTIntakeCoordinators@stanfordchildrens.org</a>	Hematologic malignancies
Phase I (NCT02963064)	<b>A Study to Evaluate the Safety and Tolerability of Tandemly Purified Allogeneic CD34+ CD90+ Hematopoietic Stem Cells (HSC) Administered Following Conditioning with Briquilimab to Achieve Engraftment and Immune Reconstitution in Patients with Severe Combined Immunodeficiency (SCID)</b>	Jasper Therapeutics	Rajni Agarwal	<a href="mailto:DL-SCTIntakeCoordinators@stanfordchildrens.org">DL-SCTIntakeCoordinators@stanfordchildrens.org</a>	Non-malignant disorders
Phase I (NCT04105166)	<b>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)</b>	Rocket Pharmaceuticals	Ami Shah	<a href="mailto:DL-SCTIntakeCoordinators@stanfordchildrens.org">DL-SCTIntakeCoordinators@stanfordchildrens.org</a>	Non-malignant disorders