Clinical Trials for High-Risk Pediatric Cancers

UPDATED SPRING 2025

Children's Hospital of Philadelphia®

Solid Tumors						
Protocol Number	**Title**	**Drug(s)**	**Diagnosis**	**Testing required for enrollment**	**Brief treatment plan**	**Clinicaltrials.gov ID**
PEPN2011	A Phase 1/2 Study of Tegavivint (IND#156033, NSC#826393) in Children, Adolescents, and Young Adults with Recurrent or Refractory Solid Tumors, Including Lymphomas and Desmoid Tumors	Tegavivint	Desmoid tumor, Ewing sarcoma, HCC, Hepatoblastoma, Non-Hodgkin Lymphoma, Osteosarcoma, Solid tumors (excluding CNS tumors), Wilm's tumor	None required. Patients with Wnt pathway aberrations are included for enrollment in part B.	Tegavivint 4-hour infusion on days 1, 8, and 15 of a 28-day cycle.	NCT04851119
22DT025	LArotrectiNib To Enhance RAI avidity in patients with differentiated thyroid cancer harboring NTRK fusions (LANTERN)	Larotrectinib	Thyroid cancer	NTRK (1-3) gene fusion identified.	28-day cycle with twice a day PO administration. Whole body scan at 4 weeks of therapy with disease assessments at the 3- and 6-month time points. After the 6 month mark, the patient will receive Thyrogen stimulated 131I therapy.	NCT05783323
22ST012	A phase I trial of Cabozantinib (XL184) in combination with high-dose Ifosfamide in adults and children with relapsed/refractory Ewing sarcoma and osteosarcoma	Cabozantinib and ifosfamide	Ewing sarcoma, Osteosarcoma	None required.	Cabozantinib will be given daily, PO for a 28-day cycle. High dose ifosfamide will be given as a continuous infusion on days 1-5 in cycle 1-4.	NCT06156410
23DT022	Selpercatinib to enhance RAI avidity in children, adolescents, and young adults with newly diagnosed differentiated thyroid cancers harboring RET fusions (RAISE trial)	Selpercatinib	Thyroid cancer	RET gene alteration identified.	28-day cycle with selpercatinib given twice daily PO. After 4 weeks of therapy, patient will have a whole-body scan. Disease assessments will occur at 3 and 6 months. After the 6-month assessment, the patient will receive Thyrogen stimulated 131I therapy.	NCT06458036
23DT024	Restoration of Radioiodine Uptake with Selpercatinib in RET Fusion-Positive Radioiodine-Refractory Thyroid Cancer: A Phase 2 Study Performed in Collaboration with the International Thyroid Oncology Group (ITOG)	Selpercatinib	Thyroid cancer	RET gene fusion identified.	28-day cycle. Administered every 12 hours PO.	NCT05668962
23DT018	An Open-Label, Multicenter, First-in-Human, Phase 1 Dose-Escalation and Multicohort Expansion Study of INBRX-109 in Subjects with Locally Advanced or Metastatic Solid Tumors, Including Sarcomas	INBRX-109 and temozolomide, with additional irinotecan in one cohort.	Ewing sarcoma, SDH-deficient solid tumors, GIST. Asymptomatic CNS metastases allowed.	Some patients require proof of loss of SDHB expression by IHC and/or assessment of SDH (A/B/C/D) gene mutation(s) by next generation sequencing.	Cohort 3: 21 day cycle. IV INBRX-109 day 1, PO temozolomide and IV irinotecan days 1-5. Cohort 5: 28 day cylce. IV INBRX-109 day 1, PO temozolomide days 1-21.	NCT03715933
23DT016	A Multi-Institution Study of TGFβ imprinted, Ex Vivo Expanded Universal Donor NK Cell Infusions as Adoptive Immunotherapy in Combination with Gemcitabine and Docetaxel in Patients with Relapsed or Refractory Pediatric Bone and Soft Tissue Sarcomas: The TiNKS Trial	TGFβ with Gemcitabine and Docetaxel	Ewing sarcoma, Non-rhabdo soft tissue sarcoma, Osteosarcoma, Rhabdomyosarcoma	None required.	21-day cycle. Lymphodepletion with gemcitabine (days 1 and 8 IV) and docetaxel (day 8 IV) followed by TGFβi NK cells infused on day 12.	NCT05634369
24DT014	A phase 1/2 clinical trial of the novel topoisomerase I inhibitor PEEL-224 as a single agent and in combination with vincristine and temozolomide in children with refractory, progressive or relapsed solid tumors	PEEL-224, vincristine, temozolomide	Solid tumors (not including CNS tumors), Neuroblastoma, Rhabdomyosarcoma	None required.	21-day cycle. PEEL-224 infused on days 1 and 8. Phase 1B and phase 2 will incluce a vincristine infusion on the same days as PEEL-224 and temozolomide on days 1-5.	NCT06721689
Inclusive of Neuro-Onc Diagnoses:						

Protocol Number	Title	Drug(s)	Diagnosis	Testing required for enrollment	Brief treatment plan	Clinicaltrials.gov ID
22DT011	Lurbinectedin in FET-Fused Tumors	Lurbinectedin	Solid tumors (including CNS tumors)	Identified FET fusion including EWSR1, FUS, or TAF15. Patients with EWS-FLI1 are eligible for dose escalation. Patients with alternative FET-ETS fusions are eligible for the exploratory cohort.	IV infusion of lurbinectedin on days 1 and 4 of a 21-day cycle.	NCT05918640
23DT011	VITAS: Atezolizumab in combination with chemotherapy for pediatric relapsed/refractory solid tumors: An open-label, phase I/II, single-arm, multi-center trial	Atezolizumab, vincristine, irinotecan, and temozolomide	Rhabdomyosarcoma, Solid tumors (including CNS tumors)	All patients will be evaluated for PD-L1 status. Some patients will be required to be positive, depending on overall enrollment of patients. Please reach out for details.	21-day cycle with infusion of all drugs on days 1-5 of each cycle.	NCT04796012
20DT016	A Phase 1/2, Open-label, Safety, Tolerability, Pharmacokinetics, and Anti-Tumor Activity Study of Repotrectinib in Pediatric and Young Adult Subjects with Advanced or Metastatic Malignancies Harboring ALK, ROS1, or NTRK1-3 Alterations (CARE)	Repotrectinib	Lymphoma, Solid tumors (including CNS tumors)	Identified NTRK (1-3) gene fusion, ROS1 gene fusion, or other ROS1 aberration.	Drug will be taken PO, daily or BID depending on dose. This follows a 28-day cycle.	NCT04094610
22DT014	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	Lurbinectedin	DSRCT, Ewing sarcoma, myxoid liposarcoma, Solid tumors (including CNS tumors)	Identified FET, FET-ETS, or EWS fusions required for some cohorts.	IV infusion given on day 1 of each cycle. 21-day cycles. CVC preferred.	NCT05734066
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	Tiragolumab and Atezolizumab	Malignant rhaboid tumor, Solid tumors (including CNS tumors) that are SMARCB1 or SMARCA4 deficient	Must have SMARCB1 (INI1) or SMARCA4 deficient tumors.	21-day cycle with drug infused on the first day of each cycle.	NCT05286801
PEPN2111	A Phase 1/2 Trial of CBL0137 (NSC# 825802, IND# 155843) in Patients with Relapsed or Refractory Solid Tumors including CNS Tumors and Lymphoma	CBL0137	Lymphoma, Solid tumors (including CNS tumors)	None required.	21-day cycle with a 30-minute infusion on days 1 and 8. Must be infused via CVC.	NCT04870944
23DT019A	LIGHTBEAM-U01 Substudy 01A: A Phase 1/2 Substudy to Evaluate the Safety and Efficacy of Zilovertamab Vedotin in Pediatric and Young Adult Participants With Hematologic Malignancies or Solid Tumors	Zilovertamab vedotin	B-ALL, DLBCL/Burkitt lymphoma, neuroblastoma, Ewing sarcoma	None required.	21-day cycle with IV administration on day 1 of each cycle. Additional intrathecal therapy will be used for patients with B-ALL and DLBCL/Burkitt lymphoma for CNS prophylaxis.	NCT06395103

Liquid Tumors

Protocol Number	Title	Drug(s)	Diagnosis	Testing required for enrollment	Brief treatment plan	Clinicaltrials.gov ID
ADVL18P1	An Open-Label Feasibility Study to Assess the Safety and Pharmacokinetics of Enasidenib in Pediatric Patients with Relapsed/Refractory Acute Myeloid Leukemia R/R-AML with an Isocitrate Dehydrogenase-2 IDH2 Mutation	Enasidenib	Acute myeloid leukemia	Identified IDH2 mutation.	28-day cycle. PO administration once daily.	NCT04203316

Protocol Number	Title	Drug(s)	Diagnosis	Testing required for enrollment	Brief treatment plan	Clinicaltrials.gov ID
PEPN2312	A Phase 1 study of GRN163L (Imetelstat, IND# 170891, NSC# 754228) in combination with fludarabine and cytarabine for patients with acute myeloid leukemia that is in second or greater relapse or that is refractory to relapse therapy; myelodysplastic syndrome or juvenile myelomonocytic leukemia in first or greater relapse or is refractory to relapse therapy	Imetelstat, Fludarabine, Cytarabine	Acute myeloid leukemia, myelodysplastic syndrome, juvenile myelomonocytic leukemia	Patients must meet standards for relapsed disease as outlined in the protocol.	IV Imetelstat on days 1 and 8 of a 28 day cycle, with fludarabine and cytarabine on days 2-6. Intrathecal cytarabine or intrathecal triples will be used as well. For patients with down syndrome, leucovorin calcium with follow the intrathecal triples therapy.	NCT06247787

Non-Therapeutic Studies

Protocol Number	Title	Drug(s)	Diagnosis	Testing required for enrollment	Brief treatment plan	Clinicaltrials.gov ID
23DT083	Synovial Sarcoma Registry and Biospecimen Repository	N/A	Synovial sarcoma	None required.	N/A	NCT05910307
PEPN22P1	A Pharmacokinetic Study of VinCRIStine in Infants Dosed According to BSA-Banded Infant Dosing Tables and Older Children Dosed by Traditional BSA Methods	N/A	Unspecified	None required.	Non-interventional study. Patient should be receiving SOC vinCRIStine at the 1.5 mg/m2 dose level to participate in PK draws.	NCT05359237
21ST081	Targeted Therapy to Increase RAI Uptake in Patients with Metastatic Differentiated Thyroid Cancer	N/A	Thyroid cancer	Identified NTRK-fusion, RET-fusion, ALK-fusion, BRAF V600 mutation, or other targetable alteration as approved by the study PI.	The patient will receive an oncogene-specific targeted therapy for 28 days (separate from this study) and then participate in a thyrogen stimulated whole body scan.	NCT05024929
20ST086	Liquid Biopsy in Ewing sarcoma and Osteosarcoma as a Prognostic and Response Diagnostic: The LEOPARD Study	N/A	Ewing sarcoma, Osteosarcoma, Primitive neuroectodermal tumors	None required.	N/A	NCT06068075
24DT081	Precision Medicine for High-Risk Pediatric Cancers: Deep Profiling and Phenotyping and Its Effect on Care (DEEP DIVE)	N/A	Rare solid tumors, including sarcomas of any subtype	None required.	N/A	N/A