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	NCT Number	Title	Other Names	Status	Conditions	Interventions	Characteristics	Population	Sponsor/Collaborators	Dates

1	NCT03861273	A Study to Evaluate the Efficacy and Safety of Factor IX Gene Therapy With PF-06838435 in Adult Males With Moderately Severe to Severe Hemophilia B Study Documents:	Title Acronym: Other Ids: C0371002 2018-003086-33 (EudraCT Number)	Active, not recruiting	Hemophilia B	Biological: PF-06838435/ fidanacogene elaparvovec Gene Therapy	Study Type: Interventional Phase: Phase 3 Study Design: Allocation: Non-Randomized Intervention Model: Single Group Assignment Masking: None (Open Label) Primary Purpose: Treatment Primary Outcome Measures: • Annualized bleeding rate (ABR) [Time Frame: First 12 months post PF 06838435 infusion] • Vector derived FIX:C level [Time Frame: Week 12 to 12 months post PF 06838435 infusion] Secondary Outcome Measures: • Annualized infusion rate (AIR) of exogenous Factor IX Activity [Time Frame: First 12 months post study drug infusion] • Annualized Factor IX Activity consumption [Time Frame: 12 months post study drug infusion] • Annualized number of bleeding events of specific type: spontaneous and traumatic, and untreated [Time Frame: 12 months post study drug infusion] • Frequency of target joint bleeds [Time Frame: 12 months post study drug infusion] • Percentage of the participants without bleeds [Time Frame: 12 months post study drug infusion] • Change in joint health as measured by the Hemophilia Joint Health Score (HJHS) instrument [Time Frame: 12 months post study drug infusion] • Patient Reported Outcome (PRO) instrument - Hemophilia Quality of Life (Haem A QoL) [Time Frame: 12 months post study drug infusion] • Patient Reported Outcome (PRO) instrument - Hemophilia Activities List (HAL) [Time Frame: 12 months post study drug infusion] • Patient Reported Outcome (PRO) instrument - Patient Global Impression of Change-Hemophilia (PGIC-H) [Time Frame: 12 months post study drug infusion] • Annualized Bleeding Rate [Time Frame: Annually for 6 years] • Vector derived Factor IX activity (FIX:C) level at steady state [Time Frame: Annually for 6 years] • Annualized infusion rate (AIR) of exogenous Factor IX [Time Frame: Annually for 6 years] • Annualized Factor IX consumption [Time Frame: Annually for 6 years] • Annualized number of bleeding events of specific type: spontaneous and traumatic, and untreated [Time Frame: Annually for 6 years] • Frequency of target joint bleeds [Time Frame: Annually for 6 years] • Patient Reported Outcome (PRO) instrument - Hemophilia Quality of Life (Haem A QoL) [Time Frame: Annually for 6 years] • Patient Reported Outcome (PRO) instrument - Hemophilia Activities List (HAL) [Time Frame: Annually for 6 years] • Patient Reported Outcome (PRO) instrument - Patient Global Impression of Change - Hemophilia (PGIC-H) [Time Frame: Annually for 6 years] • Incidence and severity of all adverse events collected during the study [Time Frame: For the duration of 6 years after PF-06838435 infusion]	Actual Enrollment: 45 Estimated Enrollment: Original Estimated Enrollment: 55 Age: 18 Years to 65 Years (Adult, Older Adult) Sex: Male	Study Sponsors: *Same as current* Collaborators: Not Provided	Study Start: July 29, 2019 Primary Completion: November 21, 2022 (Final data collection date for primary outcome measure) Study Completion: March 11, 2030 First Posted: March 4, 2019 Results First Posted: Last Update Posted: September 14, 2022

2	NCT02797470	Gene Therapy in Treating Patients With Human Immunodeficiency Virus-Related Lymphoma Receiving Stem Cell Transplant Study Documents:	Title Acronym: Other Ids: AMC-097 NCI-2015-01745 (Registry Identifier: CTRP (Clinical Trial Reporting Program)) 9933 (Other Identifier: CTRP (Clinical Trial Reporting Program)) AMC 097 (Other Identifier: AIDS Malignancy Consortium) 097 (Other Identifier: AIDS Malignancy Consortium) AMC-097 (Other Identifier: CTEP) U01CA121947 (U.S. NIH Grant/Contract)	Recruiting	• HIV Infection • Mature T-Cell and NK-Cell Non-Hodgkin Lymphoma • Plasmablastic Lymphoma • Recurrent Adult Hodgkin Lymphoma • Recurrent Adult Non-Hodgkin Lymphoma • Recurrent Burkitt Lymphoma • Recurrent Follicular Lymphoma • Stage III Follicular Lymphoma • Stage III Mantle Cell Lymphoma • Stage IV Follicular Lymphoma • Stage IV Mantle Cell Lymphoma	• Procedure: Autologous Hematopoietic Stem Cell Transplantation Undergo infusion of lentivirus vector CCR5 shRNA/TRIM5alpha/TAR decoy-transduced autologous CD34-positive hematopoietic progenitor cells Other Name: Autologous Stem Cell Transplantation • Drug: Carmustine 300 mg/m2 on Day -6, as part of BEAM and R-BEAM regimens. Other Names: ○ BCNU ○ Becenum ○ Becenun ○ BiCNU ○ Bis(chloroethyl) Nitrosourea ○ Bis-Chloronitrosourea ○ Carmubris ○ Carmustin ○ Carmustinum ○ FDA 0345 ○ Gliadel ○ N,N'-Bis(2-chloroethyl)-N-nitrosourea ○ Nitrourean ○ Nitrumon ○ SK 27702 ○ SRI 1720 ○ WR-139021 • Drug: Cytarabine 100 mg/m2 BID on Days -5 through -2, as part of BEAM and R-BEAM regimens. Other Names: ○ .beta.-Cytosine arabinoside ○ 1-.beta.-D-Arabinofuranosyl-4-amino-2(1H)pyrimidinone ○ 1-.beta.-D-Arabinofuranosylcytosine ○ 1-Beta-D-arabinofuranosyl-4-amino-2(1H)pyrimidinone ○ 1-Beta-D-arabinofuranosylcytosine ○ 1.beta.-D-Arabinofuranosylcytosine ○ 2(1H)-Pyrimidinone, 4-Amino-1-beta-D-arabinofuranosyl- ○ 2(1H)-Pyrimidinone, 4-amino-1.beta.-D-arabinofuranosyl- ○ Alexan ○ Ara-C ○ ARA-cell ○ Arabine ○ Arabinofuranosylcytosine ○ Arabinosylcytosine ○ Aracytidine ○ Aracytin ○ Aracytine ○ Beta-Cytosine Arabinoside ○ CHX-3311 ○ Cytarabinum ○ Cytarbel ○ Cytosar ○ Cytosar-U ○ Cytosine Arabinoside ○ Cytosine-.beta.-arabinoside ○ Cytosine-beta-arabinoside ○ Erpalfa ○ Starasid ○ Tarabine PFS ○ U 19920 ○ U-19920 ○ Udicil ○ WR-28453 • Drug: Etoposide VP-16: 100 mg/m2 BID on Days -5 through -2, as	Study Type: Interventional Phase: Phase 1 Phase 2 Study Design: Allocation: N/A Intervention Model: Single Group Assignment Masking: None (Open Label) Primary Purpose: Treatment Primary Outcome Measures: • Efficacy of the candidate product defined as establishment of > 5% mononuclear blood cells expressing anti-HIV genes in the peripheral blood [Time Frame: 3 months post-transplant] Efficacy rates will be summarized by the proportion of participants who meet the criteria for efficacy, with 95% exact binomial CIs. • Incidence of adverse events, using the National Cancer Institute Common Terminology Criteria for Adverse Events (NCI CTCAE) version 4.0 [Time Frame: 1 month post-transplant] Defined as timely engraftment (collective establishment of a persistent absolute neutrophil count of at least 500 cells/mm^3 and platelet count of 20,000 cells/mm^3 without transfusion for 3 consecutive days) in the absence of any study candidate-specific grade 3 and 4 non-hematopoietic organ toxicity or any clonal expansion. Toxicity will be summarized as the proportion experiencing a given toxicity or group of toxicities, at or above a specified level of severity, with exact 95% confidence intervals (CIs). Secondary Outcome Measures: • CD4 recovery [Time Frame: Up to 24 months post-treatment] • Complete response rate [Time Frame: Up to 15 years] Summarized descriptively. For dichotomous endpoints, the frequency, proportion, and exact 95% confidence interval for proportion will be calculated. • Duration of complete response (CR) [Time Frame: Time from the first documentation of CR until first date that relapsed or progressive disease is objectively documented, assessed up to 15 years] Time-to-event data will be presented graphically by Kaplan-Meier plots and summarized by estimated median time to event (if that is estimable from the data) with 95% confidence interval. • Duration of gene modified HIV-1 resistant peripheral blood cells and gut mucosal immune cells [Time Frame: Up to 24 months post-transplant] Summarized descriptively. Continuous measures will be summarized by mean (SD) and median (range), with log transformation if necessary for skewed measures, as would be typical for cell counts. • Hematologic function, defined as ANC > 1500, Hb > 10 g/dl without transfusion, and platelets > 100,000 [Time Frame: Day 100] • HIV-1 viral load over time [Time Frame: Up to 24 months post-transplant] • Incidence of toxicities, infections, transfusions, and infusion-related reactions, using the NCI CTCAE version 4.0 [Time Frame: Up to 15 years] • Integration sites of vector sequences in circulating cells [Time Frame: Up to 24 months post-transplant] • Overall survival [Time Frame: Time from start of study treatment to death, assessed up to 15 years] Time-to-event data will be presented graphically by	Actual Enrollment: Estimated Enrollment: 18 Original Estimated Enrollment: *Same as current* Age: 18 Years and older (Adult, Older Adult) Sex: All	Study Sponsors: *Same as current* Collaborators: • National Cancer Institute (NCI) • California Institute for Regenerative Medicine (CIRM)	Study Start: June 23, 2016 Primary Completion: December 31, 2024 (Final data collection date for primary outcome measure) Study Completion: June 30, 2025 First Posted: June 13, 2016 Results First Posted: Last Update Posted: September 15, 2022

3	NCT04798235	First-in-Human Study of TSHA-101 Gene Therapy for Treatment of Infantile Onset GM2 Gangliosidosis Study Documents:	Title Acronym: Other Ids: TSHA-101-IST-001	Active, not recruiting	Infantile GM2 Gangliosidosis (Disorder)	Biological: TSHA-101 AAV9 viral vector containing HEXA and HEXB genes to be administered via Intrathecal injection	Study Type: Interventional Phase: Phase 1 Phase 2 Study Design: Allocation: N/A Intervention Model: Single Group Assignment Masking: None (Open Label) Primary Purpose: Treatment Primary Outcome Measures: *Same as current* Secondary Outcome Measures: • Safety and tolerability: Viral shedding analysis [Time Frame: 1 year] Positive presence of viral DNA from biological fluids (whole blood, urine, saliva, and stool) • Assessment of Immunogenicity: Biomarkers in serum milestones [Time Frame: 1 year] Summary of neutralizing antibodies (NAbs) titers for adeno-associated virus, serotype 9 (AAV9) and Hex A • Assessment of Immunogenicity: Biomarkers in serum [Time Frame: 1 year] Summary of total antibodies (TAbs) titers for AAV9 and Hex A • Assessment of Immunogenicity: Biomarkers in peripheral blood mononuclear cells (PBMCs [Time Frame: 5 years] Summary of PBMCs for enzyme-linked immune absorbent spot (ELISpot) assays for cytokine secretion against AAV9 and Hex A • Overall Survival [Time Frame: treatment to death from any cause, up to 5 years] Estimated using the Kaplan-Meier method • Hex A Enzyme Activity: Cerebrospinal fluid (CSF) and serum [Time Frame: 1 year] Change from baseline • Head Control: Number of events for abnormal head control [Time Frame: 1 year] change from Baseline • Change from Baseline in motor function: Children's Hospital of Philadelphia Infant Test of Neuromuscular Disorders (CHOP-INTEND) [Time Frame: 1 year] The test consists of 16 items (body parts), where each item is tested for both sides of the body, left and right. The best score is taken for each item (with a maximum score of 4), and the scores are summed over all 16 items with a possible total CHOP-INTEND score of 64. • Change from Baseline in Motor Function: Modified Ashworth Scale [Time Frame: 1 year] change from Baseline. Increase or decrease of muscle tone will be measured by the Modified Ashworth Scale. Frequency counts and percentages will be presented by score (0, 1, 1+, 2, 3, and 4), muscle, side, and visit for the safety population. Flexion and extension of the knee and elbow will be measured on both sides, along with hip adduction and abduction on both sides of the body. • Clinical Efficacy Assessment: Progression of Hypotonia [Time Frame: 1 year] Assessed through neurological examinations as present or absent. Baseline to each post-Baseline visit • Clinical Efficacy Assessment: Dysphagia [Time Frame: From onset up to 3 years, if present] Assessment of the dysphagia events- assessed as present or absent.	Actual Enrollment: 3 Estimated Enrollment: Original Estimated Enrollment: 6 Age: up to 15 Months (Child) Sex: All	Study Sponsors: *Same as current* Collaborators: • Taysha Gene Therapies, Inc. • GlycoNet	Study Start: March 12, 2021 Primary Completion: March 12, 2023 (Final data collection date for primary outcome measure) Study Completion: March 12, 2027 First Posted: March 15, 2021 Results First Posted: Last Update Posted: September 14, 2022

4	NCT02122952	Gene Transfer Clinical Trial for Spinal Muscular Atrophy Type 1 Study Documents:	Title Acronym: Other Ids: AVXS-101-CL-101 COAV101A12101 (Other Identifier: Novartis Pharmaceuticals)	Completed	Spinal Muscular Atrophy 1	Biological: AVXS-101 Self-complementary AAV9 carrying the SMN gene under the control of a hybrid CMV enhancer/chicken--actin promoter Other Name: Zolgensma	Study Type: Interventional Phase: Phase 1 Study Design: Allocation: Non-Randomized Intervention Model: Single Group Assignment Masking: None (Open Label) Primary Purpose: Treatment Primary Outcome Measures: Safety Outcome Measure [Time Frame: 2 years] Any one Grade III or higher treatment-related toxicity Secondary Outcome Measures: • Mortality [Time Frame: 2 years] Time from birth to time of death • Time-to-Event Outcome Measure [Time Frame: 2 years] Time from birth to medically prescribed respiratory assistance required 16 hours per day or more.	Actual Enrollment: 15 Estimated Enrollment: Original Estimated Enrollment: 9 Age: up to 6 Months (Child) Sex: All	Study Sponsors: Jerry R. Mendell Collaborators: Not Provided	Study Start: May 5, 2014 Primary Completion: December 15, 2017 (Final data collection date for primary outcome measure) Study Completion: December 15, 2017 First Posted: May 10, 2019 Results First Posted: May 10, 2019 Last Update Posted: September 15, 2022
5	NCT05139316	A Study of Adeno-Associated Virus Serotype 8-Mediated Gene Transfer of Glucose-6-Phosphatase in Patients With Glycogen Storage Disease Type Ia (GSDIa) Study Documents:	Title Acronym: Other Ids: DTX401-CL301 2020-004184-12 (EudraCT Number)	Recruiting	Glycogen Storage Disease Type IA	• Genetic: DTX401 nonreplicating, recombinant, adeno-associated virus (AAV) serotype 8 (AAV8) • Other: Placebo Normal Saline infusion • Drug: Oral corticosteroids Participants who receive DTX401 solution will receive oral corticosteroids Other Name: prednisolone • Drug: Placebo for oral corticosteroids Participants who receive Placebo will receive placebo oral corticosteroids to maintain the study blind	Study Type: Interventional Phase: Phase 3 Study Design: Allocation: Randomized Intervention Model: Crossover Assignment Masking: Quadruple (Participant, Care Provider, Investigator, Outcomes Assessor) Primary Purpose: Treatment Primary Outcome Measures: *Same as current* Secondary Outcome Measures: *Same as current*	Actual Enrollment: Estimated Enrollment: 50 Original Estimated Enrollment: *Same as current* Age: 8 Years and older (Child, Adult, Older Adult) Sex: All	Study Sponsors: *Same as current* Collaborators: Not Provided	Study Start: November 8, 2021 Primary Completion: April 2023 (Final data collection date for primary outcome measure) Study Completion: April 2024 First Posted: December 1, 2021 Results First Posted: Last Update Posted: September 19, 2022

6	NCT05541627	PhI/II Dose-Finding Study to Evaluate BV-101 Striatal Administration in Adults With Early Manifest Huntington's Disease Study Documents:	Title Acronym: Other Ids: ASK-HD-01-CS-101	Not yet recruiting	Huntington Disease	Genetic: BV-101 Gene Therapy One-time intracerebral bilateral injections of BV-101 (AAVrh10.CAG.hCYP46A1), an adeno-associated viral vector serotype Rh10 containing the human cholesterol 24-hydroxylase gene Other Name: AAVrh10.CAG.hCYP46A1	Study Type: Interventional Phase: Phase 1 Phase 2 Study Design: Allocation: Non-Randomized Intervention Model: Single Group Assignment Masking: None (Open Label) Primary Purpose: Treatment Primary Outcome Measures: *Same as current* Secondary Outcome Measures: *Same as current*	Actual Enrollment: Estimated Enrollment: 18 Original Estimated Enrollment: *Same as current* Age: 18 Years to 65 Years (Adult, Older Adult) Sex: All	Study Sponsors: *Same as current* Collaborators: Not Provided	Study Start: October 15, 2022 Primary Completion: December 31, 2025 (Final data collection date for primary outcome measure) Study Completion: December 31, 2029 First Posted: September 15, 2022 Results First Posted: Last Update Posted: September 15, 2022
7	NCT03588299	Study to Test the Safety and How Well Patients With Severe Hemophilia A Respond to Treatment With BAY 2599023 (DTX 201), a Drug Therapy That Delivers a Healthy Version of the Defective Factor VIII Gene Into the Nucleus of Liver Cells Using an Altered, Non-infectious Virus (AAV) as a "Shuttle". Study Documents:	Title Acronym: Other Ids: 19429 2017-000806-39 (EudraCT Number)	Active, not recruiting	Hemophilia A	Drug: BAY2599023 (DTX201) Single escalating doses with 4 dose steps; Single intravenous (IV) administration.	Study Type: Interventional Phase: Phase 1 Phase 2 Study Design: Allocation: N/A Intervention Model: Single Group Assignment Masking: None (Open Label) Primary Purpose: Treatment Primary Outcome Measures: Number of patients with adverse events (AEs), treatment-emergent adverse events (TEAEs), serious adverse events (SAEs) and AEs/SAEs of special interest [Time Frame: Up to 52 weeks] Secondary Outcome Measures: Change of FVIII activity from baseline throughout the study [Time Frame: Up to 5 years] FVIII activity will be determined using both a one-stage assay and chromogenic assay.	Actual Enrollment: 11 Estimated Enrollment: Original Estimated Enrollment: 18 Age: 18 Years and older (Adult, Older Adult) Sex: Male	Study Sponsors: *Same as current* Collaborators: Ultragenix pharmaceutical	Study Start: November 7, 2018 Primary Completion: November 3, 2026 (Final data collection date for primary outcome measure) Study Completion: November 30, 2026 First Posted: July 17, 2018 Results First Posted: Last Update Posted: September 14, 2022

8	NCT04055090	Extension of Phase 3 Gene Therapy for Painful Diabetic Neuropathy Study Documents:	Title Acronym: Other Ids: VMDN-003b	Completed	• Painful Diabetic Neuropathy • Diabetic Neuropathy, Painful	• Genetic: Long-Term Follow-Up of Patients who Received Engensis (VM202) No study drug is administered in this study. Patients who received Engensis (VM202) in a previous trial will be evaluated in this trial for long-term safety and efficacy. • Drug: Long-Term Follow-Up of Patients who Received Placebo No study drug is administered in this study. Patients who received Placebo in a previous trial will be evaluated in this trial for long-term safety and efficacy.	Study Type: Interventional Phase: Phase 3 Study Design: Allocation: Randomized Intervention Model: Parallel Assignment Intervention Model Description: Long term, prospective, non-interventional, safety extension study of phase 3 trial. Double blind, randomized, placebo-controlled, multicenter study/ Masking: Quadruple (Participant, Care Provider, Investigator, Outcomes Assessor) Masking Description: Double-blind Primary Purpose: Treatment Primary Outcome Measures: the difference in long-term safety [Time Frame: Baseline through Day 365 follow up] defined as occurrence of adverse events - observed between subjects receiving VM202 versus subjects receiving placebo in the VMDN-003 study Secondary Outcome Measures: • The change in the average 24-hour pain score from baseline to the Day 365 follow-up [Time Frame: baseline to the Day 365 follow-up] The change in the average 24-hour pain score from baseline to the Day 365 follow-up from the Daily Pain and Sleep Interference Diary • The change in the average 24-hour pain score from Day 270 to the Day 365 follow-up [Time Frame: Day 270 to the Day 365 follow-up] The change in the average 24-hour pain score from Day 270 to the Day 365 follow-up from the Daily Pain and Sleep Interference Diary; • Patient's Global Impression of Change (PGIC) at the Day 365 follow-up [Time Frame: At the Day 365 follow-up] The patient's global impression of change	Actual Enrollment: 101 Estimated Enrollment: Original Estimated Enrollment: 120 Age: 18 Years to 75 Years (Adult, Older Adult) Sex: All	Study Sponsors: *Same as current* Collaborators: Not Provided	Study Start: February 4, 2019 Primary Completion: July 24, 2019 (Final data collection date for primary outcome measure) Study Completion: July 24, 2019 First Posted: August 13, 2019 Results First Posted: Last Update Posted: September 14, 2022

9	NCT04281485	Study to Evaluate the Safety and Efficacy of PF-06939926 for the Treatment of Duchenne Muscular Dystrophy Study Documents:	Title Acronym: Other Ids: C3391003 2019-002921-31 (EudraCT Number)	Recruiting	Duchenne Muscular Dystrophy	• Genetic: PF-06939926 PF-06939926 will be administered as a single IV infusion at Year 1 for Cohort 1. • Other: Placebo Placebo will be administered as a single IV infusion at Year 1 for Cohort 2. • Other: Placebo Placebo will be administered as a single IV infusion at Year 2 for Cohort 1. • Genetic: PF-06939926 PF-06939926 will be administered as a single IV infusion at Year 2 for Cohort 2	Study Type: Interventional Phase: Phase 3 Study Design: Allocation: Randomized Intervention Model: Parallel Assignment Intervention Model Description: Parallel up to the measurement of the primary outcome at Week 52. At the beginning of study Year 2 participants who were originally assigned to placebo will have the opportunity to receive PF-06939926. All participants will be followed for 5 years following treatment with PF-06939926. Masking: Quadruple (Participant, Care Provider, Investigator, Outcomes Assessor) Masking Description: The study will be quadruple blind. Primary Purpose: Treatment Primary Outcome Measures: *Same as current* Secondary Outcome Measures: *Same as current*	Actual Enrollment: Estimated Enrollment: 99 Original Estimated Enrollment: *Same as current* Age: 4 Years to 7 Years (Child) Sex: Male	Study Sponsors: *Same as current* Collaborators: Not Provided	Study Start: November 5, 2020 Primary Completion: January 30, 2024 (Final data collection date for primary outcome measure) Study Completion: January 29, 2029 First Posted: February 24, 2020 Results First Posted: Last Update Posted: September 14, 2022
10	NCT04903288	A Study of SmartFlow® Magnetic Resonance (MR) Compatible Ventricular Cannula for Administering Eladocagene Exuparvovec to Pediatric Participants Study Documents:	Title Acronym: Other Ids: PTC-AADC-GT-002	Recruiting	AADC Deficiency	Genetic: Eladocagene Exuparvovec Four 0.08 milliliters (mL) infusions at a dose of 0.45×10^11 vg and a volume of 80 microliters (l) per site to 4 sites (2 per putamen), for the total dose of 1.8×10^11 vg and a total volume of 320 l per participant.	Study Type: Interventional Phase: Phase 2 Study Design: Allocation: N/A Intervention Model: Single Group Assignment Masking: None (Open Label) Primary Purpose: Treatment Primary Outcome Measures: *Same as current* Secondary Outcome Measures: *Same as current*	Actual Enrollment: Estimated Enrollment: 3 Original Estimated Enrollment: *Same as current* Age: 1 Year to 17 Years (Child) Sex: All	Study Sponsors: *Same as current* Collaborators: Not Provided	Study Start: May 12, 2021 Primary Completion: July 15, 2023 (Final data collection date for primary outcome measure) Study Completion: July 15, 2023 First Posted: May 26, 2021 Results First Posted: Last Update Posted: September 16, 2022

11	NCT01621581	AAV2-GDNF for Advanced Parkinson s Disease Study Documents:	Title Acronym: Other Ids: 120137 12-N-0137	Completed	Parkinson's Disease	Genetic: Convection enhanced delivery/AAV2-GDNF Adeno-Associated Virus Encoding Glial Cell Line-Derived Neurotrophic Factor (AAV2-GDNF) Administered via Bilateral Stereotactic Convection-Enhanced Delivery	Study Type: Interventional Phase: Phase 1 Study Design: Allocation: Non-Randomized Intervention Model: Single Group Assignment Masking: None (Open Label) Primary Purpose: Treatment Primary Outcome Measures: Assess the safety and tolerability of 4 different dose levels of AAV2-GDNF Secondary Outcome Measures: Obtain preliminary data regarding the potential for clinical responses of the 4 dose levels tested by assessing the magnitude and variability of any treatment effects (via clinical, laboratory and neuroimaging studies).	Actual Enrollment: 25 Estimated Enrollment: Original Estimated Enrollment: 28 Age: 18 Years and older (Adult, Older Adult) Sex: All	Study Sponsors: *Same as current* Collaborators: Not Provided	Study Start: March 13, 2013 Primary Completion: February 4, 2022 (Final data collection date for primary outcome measure) Study Completion: February 4, 2022 First Posted: June 18, 2012 Results First Posted: Last Update Posted: September 19, 2022
12	NCT00001405	Recruitment and Apheresis Collection of Peripheral Blood Hematopoietic Stem Cells, Mononuclear Cells and Granulocytes Study Documents:	Title Acronym: Other Ids: 940073 94-I-0073	Recruiting	• Granuloma • Granulomatous Disease, Chronic • Leukocyte Disease • Genetic Disease, X-Linked • Genetic Disease, Inborn	Not Provided	Study Type: Observational Phase: Study Design: Observational Model: Cohort Time Perspective: Other Primary Outcome Measures: Not Provided Secondary Outcome Measures: Not Provided	Actual Enrollment: Estimated Enrollment: 850 Original Estimated Enrollment: Age: 18 Years to 70 Years (Adult, Older Adult) Sex: All	Study Sponsors: *Same as current* Collaborators: Not Provided	Study Start: February 27, 1994 Primary Completion: Not Provided Study Completion: Not Provided First Posted: November 4, 1999 Results First Posted: Last Update Posted: September 19, 2022

13	NCT03602612	T Cells Expressing a Novel Fully-Human Anti-BCMA CAR for Treating Multiple Myeloma Study Documents:	Title Acronym: Other Ids: 180125 18-C-0125	Active, not recruiting	• Myeloma-Multiple • Myeloma, Plasma-Cell	• Drug: Cyclophosphamide 300 mg/m^2 IV over 30 minutes on days -5, -4, and -3 • Drug: Fludarabine 30 mg/m^2 IV infusion over 30 minutes administered immediately following the cyclophosphamide on day -5, -4, -3 • Biological: Anti-BCMA CAR T cells 0.75x10^6 - 12.0X10^6 CAR+ T cells per kg of recipient bodyweight one time dose on day 0	Study Type: Interventional Phase: Phase 1 Study Design: Allocation: Non-Randomized Intervention Model: Sequential Assignment Masking: None (Open Label) Primary Purpose: Treatment Primary Outcome Measures: *Same as current* Secondary Outcome Measures: Not Provided	Actual Enrollment: 35 Estimated Enrollment: Original Estimated Enrollment: 42 Age: 18 Years to 73 Years (Adult, Older Adult) Sex: All	Study Sponsors: *Same as current* Collaborators: Not Provided	Study Start: September 14, 2018 Primary Completion: January 1, 2023 (Final data collection date for primary outcome measure) Study Completion: January 1, 2024 First Posted: July 27, 2018 Results First Posted: Last Update Posted: September 19, 2022
14	NCT02935257	Immunotherapy for High Risk/Relapsed CD19+ Acute Lymphoblastic Leukaemia, B-cell Non-Hodgkin's Lymphoma (B-NHL) and Chronic Lymphocytic Leukaemia (CLL)/ Small Lymphocytic Lymphoma (SLL) Using CAR T-cells to Target CD19 Study Documents:	Title Acronym: Other Ids: UCL/16/0530	Recruiting	Leukemia, Lymphoblastic, Acute, Lymphoma	Biological: CD19CAT-41BBZ CAR T-cells Infusion with CD19CAT-41BBZ CAR T-cells	Study Type: Interventional Phase: Phase 1 Study Design: Allocation: N/A Intervention Model: Single Group Assignment Masking: None (Open Label) Primary Purpose: Treatment Primary Outcome Measures: • Toxicity evaluated by the incidence of grade 3-5 toxicity causally related to the ATIMP [Time Frame: 30 days] Toxicity following CD19CAR T-cell administration as evaluated by the incidence of grade 3-5 toxicity causally related to the ATIMP. • Feasibility of manufacturing CD19CAR T-cells evaluated by the number of therapeutic products generated [Time Frame: 30 days] Feasibility of adequate leucapheresis collection and generation of CAR19 T cells as evaluated by the number of therapeutic products generated. Secondary Outcome Measures: Not Provided	Actual Enrollment: Estimated Enrollment: 60 Original Estimated Enrollment: 20 Age: 16 Years and older (Child, Adult, Older Adult) Sex: All	Study Sponsors: *Same as current* Collaborators: Not Provided	Study Start: September 29, 2017 Primary Completion: December 2024 (Final data collection date for primary outcome measure) Study Completion: December 2033 First Posted: October 17, 2016 Results First Posted: Last Update Posted: September 19, 2022

15	NCT00895271	Establishing Fibroblast-Derived Cell Lines From Skin Biopsies of Patients With Immunodeficiency or Immunodysregulation Disorders Study Documents:	Title Acronym: Other Ids: 090133 09-I-0133	Enrolling by invitation	• Primary Immunodeficiency • DOCK8 • Virus Susceptibility	Not Provided	Study Type: Observational Phase: Study Design: Observational Model: Cohort Time Perspective: Cross-Sectional Primary Outcome Measures: Not Provided Secondary Outcome Measures: Not Provided	Actual Enrollment: Estimated Enrollment: 200 Original Estimated Enrollment: Age: 2 Years to 85 Years (Child, Adult, Older Adult) Sex: All	Study Sponsors: *Same as current* Collaborators: Not Provided	Study Start: June 10, 2009 Primary Completion: Not Provided Study Completion: Not Provided First Posted: May 8, 2009 Results First Posted: Last Update Posted: September 19, 2022
16	NCT02830724	Administering Peripheral Blood Lymphocytes Transduced With a CD70-Binding Chimeric Antigen Receptor to People With CD70 Expressing Cancers Study Documents:	Title Acronym: Other Ids: 160131 16-C-0131	Recruiting	• Pancreatic Cancer • Renal Cell Cancer • Breast Cancer • Melanoma • Ovarian Cancer	• Drug: Cyclophosphamide For Phase I, Days -7 and -6: Dose Level 1: 15 mg/kg/day x 2 days IV Dose Level 2: 15 mg/kg/day x 2 days IV Dose Level 3: 15 mg/kg/day x 2 days IV Dose Level 4: 15 mg/kg/day x 2 days IV Dose Level 5: 30 mg/kg/day x 2 days IV Dose Level 6: 60 mg/kg/day x 2 days IV For Phase II, Days -7 and -6: 60 mg/kg/day x 2 days IV • Drug: Fludarabine For Phase I, Days -7 to -5: Dose Level 1: 25 mg/m(2)/day x 3 days IVPB Dose Level 2: 25 mg/m(2)/day x 3 days IVPB Dose Level 3: 25 mg/m(2)/day x 3 days IVPB Dose Level 4: 25 mg/m(2)/day x 3 days IVPB Dose Level 5: 25 mg/m(2)/day x 5 days IVPB Dose Level 6: 25 mg/m(2)/day x 5 days IVPB For Phase II, Days -7 to -3: 25 mg/m(2)/day x 5 days IVPB • Drug: Aldesleukin Aldeskeukin 720,000 IU/kg IV (based on total body weight) over 15 minutes approximately every 8 hours beginning within 24 hours of cell infusion and continuing for up to 3 days (maximum 9 doses). • Biological: Anti-hCD70 CAR transduced PBL Day 0: Cells will be infused intravenously on the Patient Care Unit over 20-30 minutes (2-5 days after the last dose of fludarabine).	Study Type: Interventional Phase: Phase 1 Phase 2 Study Design: Allocation: Non-Randomized Intervention Model: Sequential Assignment Masking: None (Open Label) Primary Purpose: Treatment Primary Outcome Measures: To determine the safety of administering PBL transduced with this anti-CD70 CAR in concert with preparative lymphodepletion and high dose interleukin-2 (IL-2; aldesleukin) and to mediate regression. [Time Frame: Approximately 5 years] Secondary Outcome Measures: • Determine the in vivo survival of anti-hCD70 CAR transduced cells [Time Frame: Approximately 5 years] • Determine the toxicity of this treatment regimen [Time Frame: Approximately 5 years]	Actual Enrollment: Estimated Enrollment: 124 Original Estimated Enrollment: 113 Age: 18 Years to 70 Years (Adult, Older Adult) Sex: All	Study Sponsors: *Same as current* Collaborators: Not Provided	Study Start: April 6, 2017 Primary Completion: January 1, 2027 (Final data collection date for primary outcome measure) Study Completion: January 1, 2028 First Posted: July 13, 2016 Results First Posted: Last Update Posted: September 19, 2022

17	NCT05540964	An Antiretroviral Treatment Interruption(ATI) Study to Evaluate the Impact of AGT103-T to Suppress Human Immunodeficiency Virus Replication in the Absence of Antiretroviral Therapy Study Documents:	Title Acronym: Other Ids: AGT-HC-169	Enrolling by invitation	HIV	Other: Antiretroviral Therapy Interruption(ATI) Study participant that were previously infused with autologous genetically modified cell product will be taken off ART and followed closely by monitoring HIV rebound.	Study Type: Interventional Phase: Phase 1 Study Design: Allocation: N/A Intervention Model: Single Group Assignment Intervention Model Description: All study participant that consent to the study will be withdrawn from their Antiretroviral Therapy(ART) and monitored closely by clinic visit and laboratory testing of blood sample collected during each visit. Masking: None (Open Label) Primary Purpose: Diagnostic Primary Outcome Measures: *Same as current* Secondary Outcome Measures: *Same as current*	Actual Enrollment: Estimated Enrollment: 7 Original Estimated Enrollment: *Same as current* Age: 18 Years and older (Adult, Older Adult) Sex: All	Study Sponsors: *Same as current* Collaborators: Not Provided	Study Start: July 19, 2022 Primary Completion: July 19, 2025 (Final data collection date for primary outcome measure) Study Completion: July 19, 2025 First Posted: September 15, 2022 Results First Posted: Last Update Posted: September 15, 2022
18	NCT03190941	Administering Peripheral Blood Lymphocytes Transduced With a Murine T-Cell Receptor Recognizing the G12V Variant of Mutated RAS in HLA-A*11:01 Patients Study Documents:	Title Acronym: Other Ids: 170113 17-C-0113	Recruiting	• Pancreatic Cancer • Gastric Cancer • Gastrointestinal Cancer • Colon Cancer • Rectal Cancer	• Drug: Cyclophosphamide Days -7 and -6: Cyclophosphamide 60 mg/kg/day x 2 days IV in 250 mL D5W infused simultaneously with mesna 15 mg/kg/day over 1 hour x 2 days. • Drug: Fludarabine Days -7 to -3: Fludarabine 25 mg/m2/day IVPB daily over 30 minutes for 5 days. • Biological: Anti-KRAS G12V mTCR PBL Day 0: Cells will be infused intravenously on the Patient Care Unit over 20-30 minutes (2-4 days after the last dose of fludarabine). • Drug: Aldesleukin Aldesleukin 720,000 IU/kg IV (based on total body weight) over 15 minutes approximately every 8 hours beginning within 24 hours of cell infusion and continuing for up to 3 days (maximum 9 doses).	Study Type: Interventional Phase: Phase 1 Phase 2 Study Design: Allocation: Non-Randomized Intervention Model: Sequential Assignment Masking: None (Open Label) Primary Purpose: Treatment Primary Outcome Measures: • Response rate [Time Frame: 6 weeks (+/- 2 weeks) after cell infusion, then at week 12, every 3 months x3, every 6 months x2 years.] • Maximum Tolerated Dose [Time Frame: End of treatment] Secondary Outcome Measures: Survival and persistence of mTCR gene-engineered cells. [Time Frame: approximately 4-5 years]	Actual Enrollment: Estimated Enrollment: 110 Original Estimated Enrollment: *Same as current* Age: 18 Years to 70 Years (Adult, Older Adult) Sex: All	Study Sponsors: *Same as current* Collaborators: Not Provided	Study Start: September 21, 2017 Primary Completion: June 29, 2027 (Final data collection date for primary outcome measure) Study Completion: June 29, 2028 First Posted: June 19, 2017 Results First Posted: Last Update Posted: September 14, 2022

19	NCT05542615	Prolonged Release Pirfenidone for Advanced Residual Liver Fibrosis (MINERVA). Study Documents:	Title Acronym: Other Ids: MINERVA	Recruiting	• Liver Cirrhosis • Hepatitis C, Chronic • Epigenetic Disorder	Drug: Prolonged-Release Pirfenidone 1200 mg / day of Pirfenidone (KitosCell® LP)	Study Type: Interventional Phase: Phase 2 Study Design: Allocation: N/A Intervention Model: Single Group Assignment Intervention Model Description: This will be a real-life, open-label, proof of concept trial to assess the safety and efficacy of two daily doses of pirfenidone (KitosCell® LP), in patients with compensated liver cirrhosis. Masking: None (Open Label) Primary Purpose: Treatment Primary Outcome Measures: *Same as current* Secondary Outcome Measures: *Same as current*	Actual Enrollment: Estimated Enrollment: 60 Original Estimated Enrollment: *Same as current* Age: 18 Years and older (Adult, Older Adult) Sex: All	Study Sponsors: *Same as current* Collaborators: Hospital Central Militar CdMX	Study Start: August 1, 2019 Primary Completion: January 1, 2023 (Final data collection date for primary outcome measure) Study Completion: December 1, 2023 First Posted: September 15, 2022 Results First Posted: Last Update Posted: September 15, 2022

20	NCT04735978	Study of RP3 Monotherapy and RP3 in Combination With Nivolumab in Patients With Solid Tumours Study Documents:	Title Acronym: Other Ids: RP3-301	Recruiting	Advanced Solid Tumor	• Biological: RP3 Genetically modified HSV-1 • Biological: Nivolumab anti-PD1 monoclonal antibody	Study Type: Interventional Phase: Phase 1 Study Design: Allocation: Non-Randomized Intervention Model: Sequential Assignment Intervention Model Description: Part 1 - Dose Escalation - Participants will be enrolled into two sequential dose level cohorts. • Cohort 1: 1 × 105 plaque-forming units (PFU)/mL on Day 1 followed by 1 × 106 PFU/mL every 2 weeks (Q2W) for up to 5 doses. • Cohort 2: 1 × 106 PFU/mL on Day 1 followed by 1 × 107 PFU/mL Q2W for up to 5 doses. Part 2 - Dose Combination - Patients will be enrolled into 1 of 5 dose-expansion cohorts. Expansion Cohorts 1, 2, and 4 will enroll patients with head and neck cancer, lung cancer, breast cancer, or GI cancer. Expansion Cohort 3 will enroll patients with any solid organ malignancy who have at least 2 tumors that can be injected and biopsied. Expansion Cohort 5 will enroll patients with melanoma. • Expansion Cohort 1 (RP3 + Nivolumab) • Expansion Cohort 2 (RP3 Followed by Nivolumab) • Expansion Cohort 3 (RP3 Monotherapy Translational Cohort) • Expansion Cohort 4 (RP3 Monotherapy) • Expansion Cohort 5 (RP3 + Nivolumab in Melanoma) Masking: None (Open Label) Primary Purpose: Treatment Primary Outcome Measures: *Same as current* Secondary Outcome Measures: • Percentage of biologic activity [Time Frame: From Day 1 to 12 months following the last dose in dose escalation. From Day 1 to 100 days following the last dose in dose combination] Percentage of participants with biological activity as assessed by individual tumor responses (including erythema, necrosis, and/or inflammation and changes in tumor sizes, in injected and uninjected tumors). • Incidence of clearance of RP3 from blood and urine [Time Frame: From Day 1 to 60 days following the last dose in dose escalation. From Day 1 to 100 days following the last dose in dose combination] Incidence of clearance of RP3 from blood and urine before and after each injection • Percentage of participants with detectable RP3. [Time Frame: From Day 1 to 60 days following the last dose in dose escalation. From Day 1 to 100 days following the last dose in dose combination] Data gathered from blood, urine, swabs of injection site, dressing and oral mucosa to determine the shedding and biodistribution of RP3 • Change in HSV-1 antibody levels [Time Frame: From Day 1 to Day 43] Change in HSV-1 antibody levels during treatment compared to baseline • Percentage of HSV-1 seronegative patients with TEAEs [Time Frame: From Day 1 to 60 days following last dose in dose escalation. From Day 1 to 100 days post last dose in dose combination] Percentage of HSV-1 seronegative patients with TEAEs • Percentage of objective overall response rate (ORR) [Time Frame: Up to 3 years since first patient in] Percentage of ORR • Median duration of response [Time Frame: Up to 3	Actual Enrollment: Estimated Enrollment: 123 Original Estimated Enrollment: 48 Age: 18 Years and older (Adult, Older Adult) Sex: All	Study Sponsors: *Same as current* Collaborators: Bristol-Myers Squibb	Study Start: December 29, 2020 Primary Completion: April 2024 (Final data collection date for primary outcome measure) Study Completion: April 2024 First Posted: February 3, 2021 Results First Posted: Last Update Posted: September 16, 2022

21	NCT03374202	VRC 603: A Phase I, Dose-Escalation Study of the Safety of AAV8-VRC07 (VRC-HIVAAV070-00-GT) Recombinant AAV Vector Expressing VRC07 HIV-1 Neutralizing Antibody in Antiretroviral - Treated, HIV-1 Infected Adults With Controlled Viremia. Study Documents:	Title Acronym: Other Ids: 180030 18-I-0030	Active, not recruiting	HIV-1 Infected Adults With Controlled Viremia	Genetic: VRC-HIVAAV070-00-GT (AAV8-VRC07) AAV8-VRC07 is a recombinant AAV vector expressing a HIV-1 CD4 binding site-specific neutralizing antibody, VRC07	Study Type: Interventional Phase: Phase 1 Study Design: Allocation: N/A Intervention Model: Parallel Assignment Masking: None (Open Label) Primary Purpose: Treatment Primary Outcome Measures: • To evaluate the safety and tolerability of AAV8-VRC07 administered IM at 5x10(10) vg/kg, 5x10(11) vg/kg, or 2.5x10(12) vg/kg, to HIV-infected adults. [Time Frame: Over 52 weeks after study injection.] • To evaluate the pharmacokinetics of VRC07 at each dose level through 24 weeks after injection. [Time Frame: 24 weeks] • To determine the AAV8-VRC07 dose that achieves at least 50 mcg/Ml VRC07 concentration in serum. [Time Frame: 4 weeks post injection] Secondary Outcome Measures: • To assess for potential clinical effects of the product on CD4 cell count and viral load in study participants. [Time Frame: 24 weeks post injection] • To determine the serum concentration of VRC07 at specified time intervals for 1 year after injection, and if persistent, then every 6 months as long as there is detectable antibody in serum. [Time Frame: 1 year after injection]	Actual Enrollment: 10 Estimated Enrollment: Original Estimated Enrollment: 25 Age: 18 Years to 60 Years (Adult) Sex: All	Study Sponsors: *Same as current* Collaborators: Not Provided	Study Start: January 11, 2018 Primary Completion: August 8, 2026 (Final data collection date for primary outcome measure) Study Completion: August 8, 2026 First Posted: December 15, 2017 Results First Posted: Last Update Posted: September 19, 2022
22	NCT02062827	Genetically Engineered HSV-1 Phase 1 Study for the Treatment of Recurrent Malignant Glioma Study Documents:	Title Acronym: Other Ids: UAB-1317	Active, not recruiting	• Recurrent Glioblastoma Multiforme • Progressive Glioblastoma Multiforme • Anaplastic Astrocytoma or Gliosarcoma	Biological: M032 (NSC 733972) A single dose of HSV-1 (M032) infused through catheters into region(s) of tumor defined by MRI	Study Type: Interventional Phase: Phase 1 Study Design: Allocation: N/A Intervention Model: Single Group Assignment Masking: None (Open Label) Primary Purpose: Treatment Primary Outcome Measures: *Same as current* Secondary Outcome Measures: *Same as current*	Actual Enrollment: 24 Estimated Enrollment: Original Estimated Enrollment: 36 Age: 18 Years and older (Adult, Older Adult) Sex: All	Study Sponsors: *Same as current* Collaborators: Not Provided	Study Start: November 25, 2013 Primary Completion: September 2023 (Final data collection date for primary outcome measure) Study Completion: September 2024 First Posted: February 14, 2014 Results First Posted: Last Update Posted: September 16, 2022

23	NCT05243017	Safety and Efficacy of AMT-130 in European Adults With Early Manifest Huntington's Disease Study Documents:	Title Acronym: Other Ids: CT-AMT-130-02	Recruiting	Huntington Disease	Genetic: intra-striatal rAAV5-miHTT One time MRI-guided stereotaxic infusion of rAAV5-miHTT into the brain Other Name: AMT-130	Study Type: Interventional Phase: Phase 1 Phase 2 Study Design: Allocation: Non-Randomized Intervention Model: Sequential Assignment Intervention Model Description: The first cohort will be treated with low dose, and a total of 6 enrolled participants is anticipated. The second cohort will be treated with high dose, and a total of 9 enrolled participants is anticipated. Masking: None (Open Label) Primary Purpose: Treatment Primary Outcome Measures: *Same as current* Secondary Outcome Measures: *Same as current*	Actual Enrollment: Estimated Enrollment: 15 Original Estimated Enrollment: *Same as current* Age: 25 Years to 65 Years (Adult, Older Adult) Sex: All	Study Sponsors: *Same as current* Collaborators: Not Provided	Study Start: October 7, 2021 Primary Completion: March 2027 (Final data collection date for primary outcome measure) Study Completion: October 7, 2027 First Posted: February 16, 2022 Results First Posted: Last Update Posted: September 14, 2022

24	NCT04320888	Selpercatinib for the Treatment of Advanced Solid Tumors, Lymphomas, or Histiocytic Disorders With Activating RET Gene Alterations, a Pediatric MATCH Treatment Trial Study Documents:	Title Acronym: Other Ids: NCI-2020-01756 NCI-2020-01756 (Registry Identifier: CTRP (Clinical Trial Reporting Program)) APEC1621N (Other Identifier: Children's Oncology Group) APEC1621N (Other Identifier: CTEP) U10CA180886 (U.S. NIH Grant/Contract)	Recruiting	• Hematopoietic and Lymphoid Cell Neoplasm • Recurrent Ependymoma • Recurrent Ewing Sarcoma • Recurrent Hepatoblastoma • Recurrent Histiocytic and Dendritic Cell Neoplasm • Recurrent Langerhans Cell Histiocytosis • Recurrent Lymphoma • Recurrent Malignant Germ Cell Tumor • Recurrent Malignant Glioma • Recurrent Malignant Solid Neoplasm • Recurrent Medulloblastoma • Recurrent Neuroblastoma • Recurrent Non-Hodgkin Lymphoma • Recurrent Osteosarcoma • Recurrent Peripheral Primitive Neuroectodermal Tumor • Recurrent Rhabdoid Tumor • Recurrent Rhabdomyosarcoma • Recurrent Soft	Drug: Selpercatinib Given PO Other Names: • LOXO-292 • RET Kinase Inhibitor LOXO-292 • Retevmo • WHO 10967	Study Type: Interventional Phase: Phase 2 Study Design: Allocation: N/A Intervention Model: Single Group Assignment Masking: None (Open Label) Primary Purpose: Treatment Primary Outcome Measures: Objective response rate (complete response + partial response) in pediatric patients treated with selpercatinib (LOXO-292) [Time Frame: Up to completion of Pediatric MATCH Screening Trial (APEC1621)] Will be determined by Response Evaluation Criteria in Solid Tumors. Response rates will be calculated as the percent of evaluable patients who are responders, and confidence intervals will be constructed using the Wilson score interval method. Secondary Outcome Measures: • Progression-free survival (PFS) [Time Frame: From the initiation of subprotocol (APEC1621N) treatment to the occurrence of any of the following events: disease progression or disease recurrence or death from any cause, assessed up to completion of Pediatric MATCH Screening Trial (APEC1621)] PFS along with the confidence intervals will be estimated using the Kaplan-Meier method. • Incidence of adverse events [Time Frame: Up to completion of Pediatric MATCH Screening Trial (APEC1621)] Evaluated by Common Terminology Criteria for Adverse Events version 5. Toxicity tables will be constructed to summarize the observed incidence by type of toxicity and grade. A patient will be counted only once for a given toxicity for the worst grade of that toxicity reported for that patient. Toxicity information recorded will include the type, severity, time of onset, time of resolution, and the probable association with the study regimen.	Actual Enrollment: Estimated Enrollment: 49 Original Estimated Enrollment: *Same as current* Age: 12 Months to 21 Years (Child, Adult) Sex: All	Study Sponsors: *Same as current* Collaborators: Children's Oncology Group	Study Start: September 14, 2020 Primary Completion: September 30, 2027 (Final data collection date for primary outcome measure) Study Completion: September 30, 2027 First Posted: March 25, 2020 Results First Posted: Last Update Posted: September 19, 2022

25	NCT00389610	Adjuvant GVAX Vaccine Therapy in Patients With Pancreatic Cancer Study Documents:	Title Acronym: Other Ids: J0619 P30CA006973 (U.S. NIH Grant/Contract) JHOC-SKCCC-J0619 (JHM IRB) NA_00002731	Active, not recruiting	Pancreatic Cancer	Biological: GVAX pancreas vaccine Given intradermally Other Name: Two irradiated allogeneic pancreatic tumor cells transfected with the GM-CSF gene	Study Type: Interventional Phase: Phase 2 Study Design: Allocation: Non-Randomized Intervention Model: Parallel Assignment Masking: None (Open Label) Primary Purpose: Treatment Primary Outcome Measures: Not Provided Secondary Outcome Measures: Not Provided	Actual Enrollment: 56 Estimated Enrollment: Original Estimated Enrollment: Age: 18 Years and older (Adult, Older Adult) Sex: All	Study Sponsors: *Same as current* Collaborators: • The Skip Viragh Foundation • National Cancer Institute (NCI)	Study Start: September 11, 2006 Primary Completion: October 11, 2021 (Final data collection date for primary outcome measure) Study Completion: December 31, 2022 First Posted: September 2, 2022 Results First Posted: September 2, 2022 Last Update Posted: September 16, 2022

26	NCT04284774	Tipifarnib for the Treatment of Advanced Solid Tumors, Lymphoma, or Histiocytic Disorders With HRAS Gene Alterations, a Pediatric MATCH Treatment Trial Study Documents:	Title Acronym: Other Ids: NCI-2020-01015 NCI-2020-01015 (Registry Identifier: CTRP (Clinical Trial Reporting Program)) APEC1621M (Other Identifier: Children's Oncology Group) APEC1621M (Other Identifier: CTEP) U10CA180886 (U.S. NIH Grant/Contract)	Recruiting	• Malignant Solid Neoplasm • Recurrent Adrenal Gland Pheochromocytoma • Recurrent Ectomesenchymoma • Recurrent Ependymoma • Recurrent Ewing Sarcoma • Recurrent Hepatoblastoma • Recurrent Kidney Wilms Tumor • Recurrent Langerhans Cell Histiocytosis • Recurrent Malignant Germ Cell Tumor • Recurrent Malignant Glioma • Recurrent Medulloblastoma • Recurrent Melanoma • Recurrent Neuroblastoma • Recurrent Non-Hodgkin Lymphoma • Recurrent Osteosarcoma • Recurrent Peripheral Primitive Neuroectodermal Tumor • Recurrent Rhabdoid Tumor • Recurrent Rhabdoid Tumor of the Kidney • Recurrent Rhabdomy	Drug: Tipifarnib Given PO or via nasogastric or gastric tube Other Names: • R115777 • Zarnestra	Study Type: Interventional Phase: Phase 2 Study Design: Allocation: N/A Intervention Model: Single Group Assignment Masking: None (Open Label) Primary Purpose: Treatment Primary Outcome Measures: Objective response rate (complete response + partial response) in pediatric patients treated with tipifarnib [Time Frame: Up to 7 years] Will be determined by Response Evaluation Criteria in Solid Tumors. Response rates will be calculated as the percent of evaluable patients who are responders, and confidence intervals will be constructed using the Wilson score interval method. Secondary Outcome Measures: • Progression free survival (PFS) [Time Frame: From the initiation of subprotocol treatment, until disease progression or disease recurrence or death from any cause, assessed up to 7 years] PFS along with the confidence intervals will be estimated using the Kaplan-Meier method. • Incidence of adverse events [Time Frame: Up to 7 years] Evaluated by Common Terminology Criteria for Adverse Events version 5. Toxicity tables will be constructed to summarize the observed incidence by type of toxicity and grade. A patient will be counted only once for a given toxicity for the worst grade of that toxicity reported for that patient. Toxicity information recorded will include the type, severity, time of onset, time of resolution, and the probable association with the study regimen.	Actual Enrollment: Estimated Enrollment: 49 Original Estimated Enrollment: *Same as current* Age: 12 Months to 21 Years (Child, Adult) Sex: All	Study Sponsors: *Same as current* Collaborators: Not Provided	Study Start: July 13, 2020 Primary Completion: September 30, 2027 (Final data collection date for primary outcome measure) Study Completion: September 30, 2027 First Posted: February 26, 2020 Results First Posted: Last Update Posted: September 19, 2022