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	NCT Number	Title	Other Names	Status	Conditions	Interventions	Characteristics	Population	Sponsor/Collaborators	Dates
1	NCT02315599	Follow-Up Evaluation for Gene-Therapy-Related Delayed Adverse Events After Participation in Pediatric Oncology Branch Clinical Trials Study Documents:	Title Acronym: Other Ids: 150028 15-C-0028	Enrolling by invitation	• Pediatric Cancers • Hematologic Malignancies • Solid Tumors	Not Provided	Study Type: Observational Phase: Study Design: Observational Model: Cohort Time Perspective: Prospective Primary Outcome Measures: Conduct long term safety evaluations after gene therapy [Time Frame: Every 3 months X 1 year then annually X 15 years] Secondary Outcome Measures: Not Provided	Actual Enrollment: Estimated Enrollment: 500 Original Estimated Enrollment: *Same as current* Age: 1 Year to 99 Years (Child, Adult, Older Adult) Sex: All	Study Sponsors: *Same as current* Collaborators: Not Provided	Study Start: December 23, 2014 Primary Completion: April 1, 2035 (Final data collection date for primary outcome measure) Study Completion: August 1, 2050 First Posted: December 12, 2014 Results First Posted: Last Update Posted: September 21, 2022
2	NCT04728841	Gene Therapy for Chinese Hemophilia A Study Documents:	Title Acronym: Other Ids: IHBDH-GTHA-2020	Recruiting	• Hemophilia A • Gene Therapy	Genetic: Injection of GS001 Patients will be enrolled sequentially every 3 weeks or more between cohorts. Dose escalation may occur after a single patient has been safely dosed if the resulting FVIII activity at Week 3 is < 5 IU/dL.The dose levels are as follows: 1. 2×10^12 vg/kg 2. 6×10^12vg/kg 3. 2×10^13 vg/kg	Study Type: Interventional Phase: Not Applicable Study Design: Allocation: N/A Intervention Model: Single Group Assignment Masking: None (Open Label) Primary Purpose: Treatment Primary Outcome Measures: • Incidence of treatment- related adverse events [Time Frame: From the start of study treatment (Day 1) through up to the end of study (about 1 year)] • Change from baseline alanine aminotransferase [Time Frame: From the start of study treatment (Day 1) through up to the end of study (about 1 year)] • Change from baseline aspartate aminotransferase [Time Frame: From the start of study treatment (Day 1) through up to the end of study (about 1 year)] • Neutralized antibody against AAV capsid protein [Time Frame: From screening period through up to 1 years] Secondary Outcome Measures: • Vector- derived FVIII:C and FVIII antigen levels [Time Frame: From pre-dose phase through up to 1 years post-dose] • Vector shedding of GS001 [Time Frame: From date of infusion until the date of 3 consecutive documented negative results, assessed up to 1 year] • Annualized bleeding rate changes from baseline [Time Frame: From the beginning of elevation of FVIII level post-dose to the end of the study (about 1 year)]	Actual Enrollment: Estimated Enrollment: 3 Original Estimated Enrollment: *Same as current* Age: 18 Years and older (Adult, Older Adult) Sex: Male	Study Sponsors: *Same as current* Collaborators: Not Provided	Study Start: March 4, 2021 Primary Completion: July 31, 2023 (Final data collection date for primary outcome measure) Study Completion: July 31, 2023 First Posted: January 28, 2021 Results First Posted: Last Update Posted: September 21, 2022

	NCT Number	Title	Other Names	Status	Conditions	Interventions	Characteristics	Population	Sponsor/Collaborators	Dates
3	NCT05353647	A Gene Transfer Study Inducing Fetal Hemoglobin in Sickle Cell Disease (GRASP, BMT CTN 2001) Study Documents:	Title Acronym: Other Ids: P00038082 1OT2HL154815 (U.S. NIH Grant/Contract) CLIN2-12031 (Other Grant/Funding Number: California Institute for Regenerative Medicine)	Recruiting	Sickle Cell Disease	Biological: Autologous CD34+ HSC cells transduced with the lentiviral vector containing a shRNA targeting BCL11a A single infusion of autologous CD34+ HSC cells transduced with the lentiviral vector containing a shRNA targeting BCL11a	Study Type: Interventional Phase: Phase 2 Study Design: Allocation: N/A Intervention Model: Single Group Assignment Intervention Model Description: Open-label, non-randomized, multi-center, phase 2, single arm study Masking: None (Open Label) Primary Purpose: Treatment Primary Outcome Measures: *Same as current* Secondary Outcome Measures: *Same as current*	Actual Enrollment: Estimated Enrollment: 25 Original Estimated Enrollment: *Same as current* Age: 13 Years to 40 Years (Child, Adult) Sex: All	Study Sponsors: *Same as current* Collaborators: • National Heart, Lung, and Blood Institute (NHLBI) • California Institute for Regenerative Medicine (CIRM) • bluebird bio • Blood and Marrow Transplant Clinical Trials Network	Study Start: July 12, 2022 Primary Completion: May 2026 (Final data collection date for primary outcome measure) Study Completion: May 2026 First Posted: April 29, 2022 Results First Posted: Last Update Posted: September 21, 2022
4	NCT04046224	Dose-Ranging Study of ST-920, an AAV2/6 Human Alpha Galactosidase A Gene Therapy in Subjects With Fabry Disease Study Documents:	Title Acronym: Other Ids: ST-920-201	Recruiting	Fabry Disease	Biological: ST-920 Single dose of investigational product ST-920	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: *Same as current* Secondary Outcome Measures: Not Provided	Actual Enrollment: Estimated Enrollment: 48 Original Estimated Enrollment: 18 Age: 18 Years and older (Adult, Older Adult) Sex: All	Study Sponsors: *Same as current* Collaborators: Not Provided	Study Start: July 23, 2019 Primary Completion: December 2023 (Final data collection date for primary outcome measure) Study Completion: February 2024 First Posted: August 6, 2019 Results First Posted: Last Update Posted: September 22, 2022

	NCT Number	Title	Other Names	Status	Conditions	Interventions	Characteristics	Population	Sponsor/Collaborators	Dates
5	NCT03061201	A Study of Recombinant AAV2/6 Human Factor 8 Gene Therapy SB-525 (PF-07055480) in Subjects With Severe Hemophilia A Study Documents:	Title Acronym: Other Ids: SB-525-1603 C3731001 (Other Identifier: Alias Study Number)	Active, not recruiting	Hemophilia A	Biological: SB-525 (PF-07055480) Single dose of investigational product SB-525 (PF-07055480)	Study Type: Interventional Phase: Phase 2 Study Design: Allocation: N/A Intervention Model: Sequential Assignment Intervention Model Description: Dose selection based on safety and kinetics of circulating FVIII levels observed in previously dosed participants. Masking: None (Open Label) Masking Description: Open Label Primary Purpose: Treatment Primary Outcome Measures: • Number of treatment related adverse events as assessed by laboratory assessments and vital signs [Time Frame: Up to 3 years after SB-525 infusion] • Changes in circulating FVIII activity [Time Frame: Up to 3 years after SB-525 infusion] Secondary Outcome Measures: • Frequency of administration of FVIII replacement therapy after administration of SB-525 [Time Frame: Up to 3 years from baseline and after SB-525 infusion] • Number of bleeding episodes requiring treatment after the administration of SB-525 [Time Frame: Up to 3 years from baseline and after SB-525 infusion] • Change in the EQ-5D health outcome questionnaire [Time Frame: Up to 1 year from baseline and after SB-525 infusion] • Measurement of FVIII inhibitor level [Time Frame: Up to 3 years after SB-525 infusion] • Presence of AAV2/6 vector DNA in plasma, saliva, urine, stool and semen [Time Frame: Up to 3 years after SB-525 infusion]	Actual Enrollment: 11 Estimated Enrollment: Original Estimated Enrollment: 20 Age: 18 Years and older (Adult, Older Adult) Sex: Male	Study Sponsors: Sangamo Therapeutics Collaborators: Not Provided	Study Start: June 21, 2017 Primary Completion: July 23, 2024 (Final data collection date for primary outcome measure) Study Completion: July 23, 2024 First Posted: February 23, 2017 Results First Posted: Last Update Posted: September 21, 2022
6	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

	NCT Number	Title	Other Names	Status	Conditions	Interventions	Characteristics	Population	Sponsor/Collaborators	Dates
7	NCT05541627	A 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: Sequential Assignment Masking: None (Open Label) Primary Purpose: Treatment Primary Outcome Measures: *Same as current* Secondary Outcome Measures: • Anatomical and volumetric measures of brain regions impacted by HD as assessed by MRI [Time Frame: At Week 52] The magnitude and variability of change from baseline in anatomical and volumetric measures of brain regions impacted by HD as assessed by MRI will be measured • Composite Unified Huntington Disease Rating Scale (cUHDRS) [Time Frame: At Week 52] The change from baseline in the cUHDRS will be measured • Mutant Huntingtin protein (mHTT) [Time Frame: At Week 52] The change from baseline in mHTT in blood and cerebrospinal fluid (CSF) will be measured • Neurofilament light chain (NfL) [Time Frame: At Week 52] The change from baseline in blood and CSF NfL will be measured • 24OH cholesterol [Time Frame: At Week 52] The change from baseline in blood and CSF 24OH cholesterol will be measured • Magnetic resonance spectroscopy (MRS) metabolic profile [Time Frame: At Week 52] Change from baseline in MRS metabolic profile • Positron emission tomography (PET) fluoro-deoxyglucose (FDG) striatal profile [Time Frame: At Week 52] Change from baseline in PET FDG striatal profile	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 21, 2022

	NCT Number	Title	Other Names	Status	Conditions	Interventions	Characteristics	Population	Sponsor/Collaborators	Dates
8	NCT05096221	A Gene Transfer Therapy Study to Evaluate the Safety and Efficacy of SRP-9001 (Delandistrogene Moxeparvovec) in Participants With Duchenne Muscular Dystrophy (DMD) Study Documents:	Title Acronym: Other Ids: SRP-9001-301 2019-003374-91 (EudraCT Number)	Active, not recruiting	Duchenne Muscular Dystrophy	• Genetic: SRP-9001 Single IV infusion of SRP-9001. Other Name: delandistrogene moxeparvovec • Genetic: Placebo Single IV infusion of matching placebo.	Study Type: Interventional Phase: Phase 3 Study Design: Allocation: Randomized Intervention Model: Parallel Assignment Masking: Quadruple (Participant, Care Provider, Investigator, Outcomes Assessor) Primary Purpose: Treatment Primary Outcome Measures: *Same as current* Secondary Outcome Measures: • Part 1: Quantity of Micro-Dystrophin Protein Expression at Week 12 as Measured by Western Blot, in a Subset of Participants [Time Frame: Week 12] • Part 1: Change From Baseline in Time to Rise From the Floor, Time to Complete 100 and 10 meter Walk/Run, and the Timed Stair Ascend 4 Steps Test at Week 52 [Time Frame: Baseline, Week 52] • Part 1: Change From Baseline in Stride Velocity 95th Centile (SV95C) Measured by a Wearable Device [Time Frame: Baseline up to Week 52] • Part 1: Change from Baseline in Patient-Reported Outcomes Measurement Information (PROMIS) Score per Domain at Week 52 [Time Frame: Baseline, Week 52] PROMIS is a family of instruments developed and validated to assess health-related quality of life. Parents will be asked "Taking into account all aspects of your child's observable symptoms, physical ability, ability to perform daily activities and overall health, how would you rate the change in clinical status for your child since the study start? using the following rating scale 1=very much improved, 2=much improved, 3=minimally improved, 4=no change, 5=minimally worse, 6=much worse, and 7=very much worse." • Part 1: Number of Skills Gained or Improved at Week 52 as Measured by the NSAA [Time Frame: Baseline up to Week 52] • Number of Participants with a Treatment Emergent Adverse Event (TEAE), Serious Adverse Event (SAE), and Adverse Event of Special Interest (AESI) [Time Frame: Baseline up to Week 52]	Actual Enrollment: Estimated Enrollment: 125 Original Estimated Enrollment: 120 Age: 4 Years to 7 Years (Child) Sex: Male	Study Sponsors: *Same as current* Collaborators: Hoffmann-La Roche	Study Start: October 27, 2021 Primary Completion: October 31, 2023 (Final data collection date for primary outcome measure) Study Completion: November 30, 2024 First Posted: October 27, 2021 Results First Posted: Last Update Posted: September 22, 2022

	NCT Number	Title	Other Names	Status	Conditions	Interventions	Characteristics	Population	Sponsor/Collaborators	Dates
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 22, 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

	NCT Number	Title	Other Names	Status	Conditions	Interventions	Characteristics	Population	Sponsor/Collaborators	Dates
11	NCT04099797	C7R-GD2.CAR T Cells for Patients With GD2-expressing Brain Tumors (GAIL-B) Study Documents:	Title Acronym: Other Ids: H-45668 GAIL-B	Recruiting	• Diffuse Intrinsic Pontine Glioma • High Grade Glioma • Embryonal Tumor • Ependymal Tumor	• Genetic: (C7R)-GD2.CART cells 1. Dose level 0: 1 x 10^7 GD2.CART cells single transduced without C7R with lymphodepletion chemotherapy 2. Dose Level 1: 1 x 10^7 C7R-GD2.CART cells with lymphodepletion chemotherapy 3. Dose Level 2: 3 x 10^7 C7R-GD2.CART cells with lymphodepletion chemotherapy • Drug: Cyclophosphamide Patients at all dose levels will receive lymphodepletion chemotherapy. They will receive 2 daily doses of cyclophosphamide (500mg/m2/day) finishing at least 24 hours before T-cell infusion. The drug will be given intravenously (through an IV needle). Other Name: Cytoxan • Drug: Fludarabine Patients at all dose levels will receive lymphodepletion chemotherapy. They will receive 3 daily doses of fludarabine (30mg/m2/day) finishing at least 24 hours before T-cell infusion. The drug will be given intravenously (through an IV needle). Other Name: Fludara	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: Estimated Enrollment: 34 Original Estimated Enrollment: *Same as current* Age: 12 Months to 21 Years (Child, Adult) Sex: All	Study Sponsors: *Same as current* Collaborators: Center for Cell and Gene Therapy, Baylor College of Medicine	Study Start: February 3, 2020 Primary Completion: February 2023 (Final data collection date for primary outcome measure) Study Completion: February 2038 First Posted: September 23, 2019 Results First Posted: Last Update Posted: September 22, 2022
12	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

	NCT Number	Title	Other Names	Status	Conditions	Interventions	Characteristics	Population	Sponsor/Collaborators	Dates
13	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
14	NCT04445454	Mesenchymal Stromal Cell Therapy for Severe Covid-19 Infection Study Documents:	Title Acronym: Other Ids: TJT2012	Recruiting	Coronavirus Infection	Biological: Mesenchymal stromal cells Bone marrow collection and MSC expansion cultures will be carried out at the Laboratory of Cell and Gene Therapy (LTCG) at the University of Liège as described in IMPD and its SOPs. Other Name: MSC	Study Type: Interventional Phase: Phase 1 Phase 2 Study Design: Allocation: N/A Intervention Model: Single Group Assignment Intervention Model Description: This study is a monocentric prospective phase I/II clinical trial, aiming at evaluating the safety and efficacy of 3 intravenous administrations of BM-MSC in 20 patients with severe to critical COVID-19 pneumonia. Masking: None (Open Label) Primary Purpose: Treatment Primary Outcome Measures: *Same as current* Secondary Outcome Measures: *Same as current*	Actual Enrollment: Estimated Enrollment: 20 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: June 12, 2020 Primary Completion: September 30, 2024 (Final data collection date for primary outcome measure) Study Completion: September 30, 2024 First Posted: June 24, 2020 Results First Posted: Last Update Posted: September 21, 2022

	NCT Number	Title	Other Names	Status	Conditions	Interventions	Characteristics	Population	Sponsor/Collaborators	Dates
15	NCT04303416	Plasma Exchange With Albumin in AMN Patients Study Documents:	Title Acronym: Other Ids: XAMNPEAP2019	Completed	• Adrenomyeloneuropathy • Adrenoleukodystrophy	Drug: Albumin solution plasma exchange with albumin, one per week for one month, then one per month for 5 months Other Name: plasma exchange	Study Type: Interventional Phase: Phase 2 Phase 3 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: 5 Estimated Enrollment: Original Estimated Enrollment: *Same as current* Age: 18 Years to 65 Years (Adult, Older Adult) Sex: Male	Study Sponsors: *Same as current* Collaborators: Not Provided	Study Start: March 9, 2020 Primary Completion: February 24, 2021 (Final data collection date for primary outcome measure) Study Completion: September 13, 2021 First Posted: March 11, 2020 Results First Posted: Last Update Posted: September 22, 2022
16	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 21, 2022

	NCT Number	Title	Other Names	Status	Conditions	Interventions	Characteristics	Population	Sponsor/Collaborators	Dates
17	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
18	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

	NCT Number	Title	Other Names	Status	Conditions	Interventions	Characteristics	Population	Sponsor/Collaborators	Dates
19	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 21, 2022
20	NCT02475707	Administration of Donor MultiTAA-Specific T Cells for ALL Study Documents:	Title Acronym: Other Ids: H-37042 STELLA STELLA (Other Identifier: Baylor College of Medicine)	Active, not recruiting	Leukemia, Lymphoblastic (Acute)	Biological: MultiTAA-specific T cells The 3 dose levels are: Dose Level 1: 5 x 10e6 cells/m2; Dose Level 2: 1 x 10e7 cells/m2; Dose Level 3: 2 x 10e7 cells/m2 The T cells are given from 30 days post-HSCT. They are administered by intravenous injection over 1-10 minutes through either a peripheral or a central line. Patients being treated on Arm A (adjuvant) or Arm B (active disease) who have a partial response, complete response or stable disease, will be eligible to receive up to 6 further doses of multiTAA-specific T cells at the same dose as the initial infusions at a minimum of 4 weeks apart. Other Name: Multiple tumor-associated antigen (TAA)-specific T cells	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: *Same as current* Secondary Outcome Measures: *Same as current*	Actual Enrollment: 40 Estimated Enrollment: Original Estimated Enrollment: 28 Age: Child, Adult, Older Adult Sex: All	Study Sponsors: *Same as current* Collaborators: • Center for Cell and Gene Therapy, Baylor College of Medicine • The Methodist Hospital Research Institute	Study Start: February 2016 Primary Completion: October 29, 2019 (Final data collection date for primary outcome measure) Study Completion: October 2024 First Posted: June 19, 2015 Results First Posted: Last Update Posted: September 22, 2022

	NCT Number	Title	Other Names	Status	Conditions	Interventions	Characteristics	Population	Sponsor/Collaborators	Dates
21	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]	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

	NCT Number	Title	Other Names	Status	Conditions	Interventions	Characteristics	Population	Sponsor/Collaborators	Dates
22	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 21, 2022
23	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

	NCT Number	Title	Other Names	Status	Conditions	Interventions	Characteristics	Population	Sponsor/Collaborators	Dates
24	NCT05219162	Real-World Study on Gene Profile in Patients With Advanced NSCLC Who Progressed on First-Line Osimertinib Therapy(GPS). Study Documents:	Title Acronym: Other Ids: D5161R00037	Recruiting	Advanced NSCLC	Genetic: Gene Profile explore "Tumor tissue samples will be obtained by biopsy."	Study Type: Interventional Phase: Phase 4 Study Design: Allocation: N/A Intervention Model: Single Group Assignment Intervention Model Description: single arm all 200 Participants will be required to provide paired tissue and whole blood after disease progression following 1L Osimertinib. 200 tissue samples and 200 whole blood samples will be used to detect gene alteration by NGS, respectively. 200 tissue samples will be used to detect pathological transformation by IHC. Masking: None (Open Label) Primary Purpose: Diagnostic Primary Outcome Measures: *Same as current* Secondary Outcome Measures: *Same as current*	Actual Enrollment: Estimated Enrollment: 200 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: February 25, 2022 Primary Completion: May 29, 2023 (Final data collection date for primary outcome measure) Study Completion: May 30, 2023 First Posted: February 1, 2022 Results First Posted: Last Update Posted: September 21, 2022

	NCT Number	Title	Other Names	Status	Conditions	Interventions	Characteristics	Population	Sponsor/Collaborators	Dates
25	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	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

	NCT Number	Title	Other Names	Status	Conditions	Interventions	Characteristics	Population	Sponsor/Collaborators	Dates
26	NCT02069730	A Study of Drug Therapies for Salivary Gland Cancers Based on Testing of Genes Study Documents:	Title Acronym: Other Ids: GEMS-001	Recruiting	• Salivary Gland Cancer • Metastatic • Advanced • Recurrent	• Drug: Selinexor If no "druggable" aberrations are identified on the molecular profiling analysis, then patients will receive unmatched treatment with Selinexor, a selective inhibitor of nuclear export (SINE). Other Name: KPT-330 • Drug: EGFR or HER2 Inhibitor If specific "druggable" aberrations are identified on the molecular profiling analysis, then patients will receive matched treatment with EGFR or HER2 Inhibitor Other Name: Matched treatment • Drug: FGFR Inhibitor If specific "druggable" aberrations are identified on the molecular profiling analysis, then patients will receive matched treatment with FGFR Inhibitor Other Name: Matched treatment • Drug: C-KIT Inhibitor If specific "druggable" aberrations are identified on the molecular profiling analysis, then patients will receive matched treatment with C-KIT Inhibitor Other Name: Matched Treatment • Drug: Anti-androgen If specific "druggable" aberrations are identified on the molecular profiling analysis, then patients will receive matched treatment with Anti-androgens Other Name: Matched Treatment • Drug: NOTCH Inhibitor If specific "druggable" aberrations are identified on the molecular profiling analysis, then patients will receive matched treatment with NOTCH Inhibitor Other Name: Matched Treatment • Drug: MEK or PI3K Inhibitor If specific "druggable" aberrations are identified on the molecular profiling analysis, then patients will receive matched treatment with MEK or PI3K Inhibitor Other Name: Matched Treatment	Study Type: Interventional Phase: Not Applicable Study Design: Allocation: Non-Randomized Intervention Model: Parallel Assignment Masking: None (Open Label) Primary Purpose: Treatment Primary Outcome Measures: Number of participants with complete and partial response to unmatched therapy Selinexor compared to matched therapies [Time Frame: 4 years] Secondary Outcome Measures: • Number of participants with complete, partial and/or stable disease to unmatched therapy Selinexor compared to matched therapies [Time Frame: 4 years] • Length of time that participant's disease does not worsen [Time Frame: 6 months] • Percentage of each molecular aberrations in metastatic salivary gland tumors [Time Frame: 4 years]	Actual Enrollment: Estimated Enrollment: 200 Original Estimated Enrollment: 30 Age: 18 Years and older (Adult, Older Adult) Sex: All	Study Sponsors: *Same as current* Collaborators: Not Provided	Study Start: June 2014 Primary Completion: December 2026 (Final data collection date for primary outcome measure) Study Completion: December 2027 First Posted: February 24, 2014 Results First Posted: Last Update Posted: September 21, 2022