ClinicalTrials.gov: gene therapy | Last update posted in the last 7 days

	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	NCT01976091	A Gene Transfer Therapy Study to Evaluate the Safety of SRP-9004 (Patidistrogene Bexoparvovec) in Participants With Limb-Girdle Muscular Dystrophy, Type 2D (LGMD2D) Study Documents:	Title Acronym: Other Ids: 9004-101 5U01AR060911 (U.S. NIH Grant/Contract)	Completed	Limb-Girdle Muscular Dystrophy, Type 2D	Genetic: SRP-9004 Isolated Limb Infusion (ILI) Other Name: patidistrogene bexoparvovec	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: Safety with fewer than 2 grade 3 adverse events [Time Frame: 1 year from start] Safety with fewer than 2 grade 3 adverse events Secondary Outcome Measures: Efficacy outcome measure 6MWT [Time Frame: 2 years] 6 minute walk test (6MWT)-(primary variable to measure efficacy) Efficacy would be a significant improvement in distance walked in the 6 minute walk test.	Actual Enrollment: 6 Estimated Enrollment: Original Estimated Enrollment: *Same as current* Age: 7 Years and older (Child, Adult, Older Adult) Sex: All	Study Sponsors: Jerry R. Mendell Collaborators: Nationwide Children's Hospital	Study Start: February 1, 2015 Primary Completion: March 14, 2019 (Final data collection date for primary outcome measure) Study Completion: March 14, 2019 First Posted: April 1, 2022 Results First Posted: April 1, 2022 Last Update Posted: September 13, 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
7	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

8	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
9	NCT03636438	Long Term Follow Up to Evaluate DTX301 in Adults With Late-Onset OTC Deficiency Study Documents:	Title Acronym: Other Ids: 301OTC02 2018-000156-18 (EudraCT Number)	Active, not recruiting	Ornithine Transcarbamylase (OTC) Deficiency	Other: No Intervention No Intervention	Study Type: Observational Phase: Study Design: Observational Model: Other Time Perspective: Prospective Primary Outcome Measures: Number of Participants with Adverse Events and Serious Adverse Events [Time Frame: Up to 260 weeks following DTX301 administration] Secondary Outcome Measures: • Change from Baseline Over Time in the Ureagenesis Rate [Time Frame: Baseline (average of Screening and Day 1) up to 260 weeks following DTX301 administration] • Change from Baseline Over Time in 24-Hour Area Under the Curve for Plasma Ammonia [Time Frame: Baseline (Day 0 of Study 301OTC01) up to 260 weeks following DTX301 administration]	Actual Enrollment: 11 Estimated Enrollment: Original Estimated Enrollment: 12 Age: 18 Years and older (Adult, Older Adult) Sex: All	Study Sponsors: *Same as current* Collaborators: Not Provided	Study Start: August 30, 2018 Primary Completion: December 2027 (Final data collection date for primary outcome measure) Study Completion: December 2027 First Posted: August 17, 2018 Results First Posted: Last Update Posted: September 13, 2022

10	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

11	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
12	NCT00012545	Collection and Storage of Umbilical Cord Stem Cells for Treatment of Sickle Cell Disease Study Documents:	Title Acronym: Other Ids: 010122 01-H-0122	Recruiting	• Sickle Cell Disease • Sickle Cell Trait	Not Provided	Study Type: Observational Phase: Study Design: Observational Model: Case-Only Time Perspective: Cross-Sectional Primary Outcome Measures: Not Provided Secondary Outcome Measures: Not Provided	Actual Enrollment: Estimated Enrollment: 352 Original Estimated Enrollment: Age: 18 Years to 45 Years (Adult) Sex: All	Study Sponsors: National Institute of Diabetes and Digestive and Kidney Diseases (NIDDK) Collaborators: Not Provided	Study Start: November 1, 2001 Primary Completion: Not Provided Study Completion: Not Provided First Posted: March 12, 2001 Results First Posted: Last Update Posted: September 13, 2022

13	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
14	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

15	NCT05062980	Reqorsa (Quaratusugene Ozeplasmid) in Combination With Pembrolizumab in Previously Treated Non-Small Lung Cancer Study Documents:	Title Acronym: Other Ids: ONC-004	Recruiting	Non Small Cell Lung Cancer	• Biological: quaratusugene ozeplasmid Quaratusugene ozeplasmid is an experimental non-viral immunoogene therapy utilizing the TUSC2 gene , designed to target cancer cells by interrupting cell signaling pathways that allow cancer cells to grow, re-establishing pathways that promote cancer cell death and modulating the immune system response against cancer cells. Other Names: ○ GPX-001 ○ Reqorsa • Drug: pembrolizumab Pembrolizumab is a programmed death receptor-1 (PD-1) blocking antibody indicated for treatment of patients with metastatic NSCLC. Other Name: Keytruda • Drug: docetaxel Docetaxel is a microtubule inhibitor indicated for locally advanced or metastatic NSCLC after platinum-based chemotherapy failure. • Drug: ramucirumab Ramucirumab is a human vascular endothelial growth factor receptor 2 (VEGFR2) antagonist indicated for in combination with docetaxel for treatment of NSCLC with disease progression after platinum-based chemotherapy. Other Name: Cyramza	Study Type: Interventional Phase: Phase 1 Phase 2 Study Design: Allocation: Randomized Intervention Model: Sequential Assignment Intervention Model Description: Phase 1: 3+3 dose escalation to identify RP2D followed by a 12 patient dose expansion cohort. Phase 2: Parallel randomization in a 2:1 ratio to either Reqorsa at RP2D in combination with pembrolizumab or docetaxel +/- ramucirumab. Masking: Single (Outcomes Assessor) Masking Description: Tumor responses will be assessed centrally using RECIST 1.1 criteria by an independent radiology group blinded to treatment arm assignment. Primary Purpose: Treatment Primary Outcome Measures: • Maximum Tolerated Dose (MTD) - Phase 1 [Time Frame: up to 3 weeks] Dose limiting toxicity (DLT), defined as any Grade 3 prolonged non-hematological toxicity or Grade 4 prolonged hematological, organ or non-hematological toxicity or any Grade 3 prolonged cytokine release syndrome (CRS) or any Grade 4 CRS occurring during the first cycle of therapy and considered to be possibly, probably, or definitely related to GPX-001. • Progression-free Survival (PFS) - Phase 2 [Time Frame: 24 months] Number of months from randomization to the date of disease progression, confirmed by RECIST v1.1 criteria or to the date of death due to any cause. Secondary Outcome Measures: Not Provided	Actual Enrollment: Estimated Enrollment: 156 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: March 30, 2022 Primary Completion: May 2025 (Final data collection date for primary outcome measure) Study Completion: May 2026 First Posted: September 30, 2021 Results First Posted: Last Update Posted: September 13, 2022
16	NCT05536973	Safety and Efficacy of ADVM-022 in Treatment-Experienced Patients With Neovascular Age-related Macular Degeneration Study Documents:	Title Acronym: Other Ids: ADVM-022-11	Recruiting	Neovascular Age-related Macular Degeneration	• Genetic: ADVM-022 A single IVT injection of 2E11 vg/eye ADVM-022 dose in combination with one (1) of four (4) corticosteroid treatment regimens • Genetic: ADVM-022 A single IVT injection of 6E10 vg/eye ADVM-022 dose in combination with one (1) of four (4) corticosteroid treatment regimens	Study Type: Interventional Phase: Phase 2 Study Design: Allocation: Randomized Intervention Model: Parallel Assignment Masking: Double (Participant, Investigator) Primary Purpose: Treatment Primary Outcome Measures: *Same as current* Secondary Outcome Measures: *Same as current*	Actual Enrollment: Estimated Enrollment: 72 Original Estimated Enrollment: *Same as current* Age: 50 Years and older (Adult, Older Adult) Sex: All	Study Sponsors: *Same as current* Collaborators: Parexel	Study Start: August 23, 2022 Primary Completion: February 2024 (Final data collection date for primary outcome measure) Study Completion: February 2024 First Posted: September 13, 2022 Results First Posted: Last Update Posted: September 13, 2022

17	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
18	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

19	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
20	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

21	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
22	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

23	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
24	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

25	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