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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 8, 2022
2	NCT02473757	Gene Therapy Follow-up Protocol for People Previously Enrolled in CAR-T Cell Studies Study Documents:	Title Acronym: Other Ids: 150141 15-C-0141	Enrolling by invitation	• Lyphoma, B-Cell • Leukemia, B-cell • Multiple Myeloma • Hematologic Malignancies	Not Provided	Study Type: Observational Phase: Study Design: Observational Model: Cohort Time Perspective: Prospective Primary Outcome Measures: To provide long term follow up of patients previously enrolled on treatment protocols in the NCI ETIB Branch [Time Frame: 15 years] Secondary Outcome Measures: Not Provided	Actual Enrollment: Estimated Enrollment: 1000 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: September 24, 2015 Primary Completion: July 1, 2034 (Final data collection date for primary outcome measure) Study Completion: August 1, 2050 First Posted: June 17, 2015 Results First Posted: Last Update Posted: September 8, 2022

	NCT Number	Title	Other Names	Status	Conditions	Interventions	Characteristics	Population	Sponsor/Collaborators	Dates
3	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
4	NCT03505099	Pre-Symptomatic Study of Intravenous Onasemnogene Abeparvovec-xioi in Spinal Muscular Atrophy (SMA) for Patients With Multiple Copies of SMN2 Study Documents:	Title Acronym: Other Ids: AVXS-101-CL-304 2017-004087-35 (EudraCT Number) JapicCTI-184203 (Registry Identifier: JapicCTI) COAV101A12303 (Other Identifier: Novartis Pharmaceuticals)	Completed	Spinal Muscular Atrophy	Biological: onasemnogene abeparvovec-xioi A non-replicating recombinant AAV9 containing the complimentary deoxyribonucleic acid (cDNA) of the human SMN gene under the control of the cytomegalovirus (CMV) enhancer/chicken--actin-hybrid promoter (CB). The AAV inverted terminal repeat (ITR) has been modified to promote intramolecular annealing of the transgene, thus forming a double-stranded transgene ready for transcription. Other Name: Zolgensma	Study Type: Interventional Phase: Phase 3 Study Design: Allocation: N/A Intervention Model: Single Group Assignment Intervention Model Description: Open-label, single arm Masking: None (Open Label) Primary Purpose: Treatment Primary Outcome Measures: • 2 copies SMN2 gene: functional independent sitting [Time Frame: 18 months of age] Proportion of patients demonstrating functional independent sitting for at least 30 seconds • 3 copies of SMN2 gene: standing without support [Time Frame: 24 months of age] Proportion of patients achieving the ability to stand without support for at least three seconds • 4 copies of SMN2 gene: demonstrating motor improvements inconsistent with SMA natural history [Time Frame: 36 months of age] Proportion of patients demonstrating the ability to achieve a scaled score on Bayley V.3 Gross and Fine Motor Subtests within 1.5 standard deviations of chronological development reference standard Secondary Outcome Measures: Not Provided	Actual Enrollment: 30 Estimated Enrollment: Original Estimated Enrollment: 44 Age: up to 42 Days (Child) Sex: All	Study Sponsors: AveXis, Inc. Collaborators: PRA Health Sciences	Study Start: April 2, 2018 Primary Completion: June 15, 2021 (Final data collection date for primary outcome measure) Study Completion: June 15, 2021 First Posted: January 11, 2022 Results First Posted: January 11, 2022 Last Update Posted: September 7, 2022

	NCT Number	Title	Other Names	Status	Conditions	Interventions	Characteristics	Population	Sponsor/Collaborators	Dates
5	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
6	NCT05529342	Long-term Follow-up of Study Participant Treated With Lentiviral-Based Genetically Modified Autologous Cell Product ,AGT103-T Study Documents:	Title Acronym: Other Ids: AGT103-T-LTFU	Enrolling by invitation	HIV	Not Provided	Study Type: Observational [Patient Registry] Phase: Study Design: Observational Model: Case-Control Time Perspective: Prospective 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: August 29, 2022 Primary Completion: May 23, 2038 (Final data collection date for primary outcome measure) Study Completion: September 29, 2038 First Posted: September 7, 2022 Results First Posted: Last Update Posted: September 7, 2022

	NCT Number	Title	Other Names	Status	Conditions	Interventions	Characteristics	Population	Sponsor/Collaborators	Dates
7	NCT05429372	Study of Fordadistrogene Movaparvovec in Early Stage Duchenne Muscular Dystrophy Study Documents:	Title Acronym: Other Ids: C3391008 2021-003379-33 (EudraCT Number)	Recruiting	Muscular Dystrophy, Duchenne	Genetic: PF-06939926 All participants will receive a single dose of PF-06939926 on Day 1. Other Name: Fordadistrogene Movaparvovec	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: 10 Original Estimated Enrollment: *Same as current* Age: 2 Years to 3 Years (Child) Sex: Male	Study Sponsors: *Same as current* Collaborators: Not Provided	Study Start: August 8, 2022 Primary Completion: July 17, 2024 (Final data collection date for primary outcome measure) Study Completion: June 25, 2028 First Posted: June 23, 2022 Results First Posted: Last Update Posted: September 10, 2022
8	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

	NCT Number	Title	Other Names	Status	Conditions	Interventions	Characteristics	Population	Sponsor/Collaborators	Dates
9	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 9, 2022
10	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

	NCT Number	Title	Other Names	Status	Conditions	Interventions	Characteristics	Population	Sponsor/Collaborators	Dates
11	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
12	NCT00001405	Recruitment and Apheresis Collection of Peripheral Blood Hematopoietic Stem Cells, Mononuclear Cells and Granulocytes Study Documents:	Title Acronym: Other Ids: 940073 94-I-0073	Recruiting	• 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 10, 2022

	NCT Number	Title	Other Names	Status	Conditions	Interventions	Characteristics	Population	Sponsor/Collaborators	Dates
13	NCT01212055	Apheresis of Patients With Immunodeficiency Study Documents:	Title Acronym: Other Ids: 100201 10-C-0201	Recruiting	• LAD-1 • DOCK8 • GATA2 Deficancy	Not Provided	Study Type: Observational Phase: Study Design: Observational Model: Case-Control Time Perspective: Prospective Primary Outcome Measures: Not Provided Secondary Outcome Measures: Not Provided	Actual Enrollment: Estimated Enrollment: 6 Original Estimated Enrollment: *Same as current* Age: 18 Years to 40 Years (Adult) Sex: All	Study Sponsors: *Same as current* Collaborators: Not Provided	Study Start: November 8, 2010 Primary Completion: Not Provided Study Completion: Not Provided First Posted: September 30, 2010 Results First Posted: Last Update Posted: September 8, 2022
14	NCT05532761	Multidimensional Assessment of Quality of Life, Social and Professional Life and Care Utilization in Patients With Diffuse Large Cell B-cell Lymphoma Treated With CAR-T Cells Study Documents:	Title Acronym: Other Ids: 69HCL22_0430	Not yet recruiting	• Diffuse Large B-cell Lymphoma (DLBCL) • CAR-T Cells Treatment	Other: self-administered questionnaires In order to describe the experience of CAR-T cell therapy of DLBCL patients, a pharmaceutical follow-up is carried out the day before the injection (baseline) and at 1, 3, 6, 9, 12 and 18 months. These follow-ups consist of interviews with the patient and the delivery of self-administered questionnaires. The interviews will investigate drug consumption, the use of self-medication and complementary alternative therapies and the adverse effects of interest. The self-questionnaires will focus on exploring multidimensional quality of life, social and professional life, anxiety-depression or uncertainty tolerance through internationally validated questionnaires. No supplementary visits will be needed : interviews with the research team will occur at the end of hematologic consultations.	Study Type: Observational Phase: Study Design: Observational Model: Cohort Time Perspective: Prospective Primary Outcome Measures: *Same as current* Secondary Outcome Measures: Not Provided	Actual Enrollment: Estimated Enrollment: 30 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: September 2022 Primary Completion: March 2025 (Final data collection date for primary outcome measure) Study Completion: March 2025 First Posted: September 8, 2022 Results First Posted: Last Update Posted: September 8, 2022

	NCT Number	Title	Other Names	Status	Conditions	Interventions	Characteristics	Population	Sponsor/Collaborators	Dates
15	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 9, 2022
16	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 8, 2022

	NCT Number	Title	Other Names	Status	Conditions	Interventions	Characteristics	Population	Sponsor/Collaborators	Dates
17	NCT00001204	Cardiovascular Evaluation of Patients With High Cholesterol and Normal Volunteers Study Documents:	Title Acronym: Other Ids: 850105 85-H-0105	Completed	Homozygous Familial Hypercholesterolemic	Not Provided	Study Type: Observational Phase: Study Design: Observational Model: Cohort Time Perspective: Prospective Primary Outcome Measures: Not Provided Secondary Outcome Measures: Not Provided	Actual Enrollment: 73 Estimated Enrollment: Original Estimated Enrollment: Age: 2 Years to 70 Years (Child, Adult, Older Adult) Sex: All	Study Sponsors: *Same as current* Collaborators: Not Provided	Study Start: January 7, 1992 Primary Completion: Not Provided Study Completion: Not Provided First Posted: November 4, 1999 Results First Posted: Last Update Posted: September 8, 2022
18	NCT03354390	HERV-E TCR Transduced Autologous T Cells in People With Metastatic Clear Cell Renal Cell Carcinoma Study Documents:	Title Acronym: Other Ids: 180012 18-H-0012	Recruiting	Kidney Cancer	Biological: cell infusion This is a single-arm, phase 1 trial of HERV-E TCR transduced CD8+/CD34+ T cells in HLA-A*11:01 positive patients with metastatic ccRCC. The study is planned based on a Phase 1 3+3 dose escalation design. The maximum tolerated dose (MTD) is defined as the highest dose at which 0 or 1 patient in six has experienced a dose limiting toxicity (DLT). Patients with evaluable advanced/metastatic ccRCC will be recruited in up to 4 dose levels.	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 [Time Frame: 21 days] Secondary Outcome Measures: *Same as current*	Actual Enrollment: Estimated Enrollment: 24 Original Estimated Enrollment: *Same as current* Age: 18 Years to 75 Years (Adult, Older Adult) Sex: All	Study Sponsors: *Same as current* Collaborators: Loyola University Medical Center (LUMC)	Study Start: July 20, 2018 Primary Completion: April 30, 2024 (Final data collection date for primary outcome measure) Study Completion: December 31, 2032 First Posted: November 28, 2017 Results First Posted: Last Update Posted: September 10, 2022

	NCT Number	Title	Other Names	Status	Conditions	Interventions	Characteristics	Population	Sponsor/Collaborators	Dates
19	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 13, 2022

	NCT Number	Title	Other Names	Status	Conditions	Interventions	Characteristics	Population	Sponsor/Collaborators	Dates
20	NCT04875754	A Study Evaluating the Safety, Tolerability, and Range of Biologically Active Doses of ICM-203 in Mild to Moderate Knee Osteoarthritis Study Documents:	Title Acronym: Other Ids: ICM 20-1001	Recruiting	Osteoarthritis, Knee	• Genetic: ICM-203 Intra-articular injection • Drug: Placebo (saline solution) Intra-articular injection	Study Type: Interventional Phase: Phase 1 Phase 2 Study Design: Allocation: Randomized Intervention Model: Sequential Assignment Intervention Model Description: • Group 1: ICM-203 6x10e12 vg or Placebo • Group 2: ICM-203 2x10e13 vg or Placebo • Group 3: ICM-203 6x10e13 vg or Placebo Masking: Quadruple (Participant, Care Provider, Investigator, Outcomes Assessor) Primary Purpose: Treatment Primary Outcome Measures: *Same as current* Secondary Outcome Measures: • Knee pain [Time Frame: Up to Week 52] Evaluation of change from baseline in knee pain as measured using a Numerical Rating Scale (NRS) ranging from 0 (no pain) to 10 (worst pain imaginable) • Knee function [Time Frame: Up to Week 52] Evaluation of change from baseline in knee function as measured using the Function in Daily Living subscore of the Knee Injury and Osteoarthritis Outcome Score (KOOS) • Articular cartilage grade [Time Frame: Up to Week 52] Evaluation of change from baseline in articular cartilage grade as measured using MRI Osteoarthritis Knee Score (MOAKS) • Joint space width [Time Frame: Up to Week 52] Evaluation of change from baseline in Joint space width in mm as measured on knee radiograph • Humoral response to AAV5.2 capsid [Time Frame: Up to Week 52] Evaluation of change from baseline in neutralizing antibody titers against AAV5.2 in serum • Cellular immune response to AAV5.2 capsid [Time Frame: Up to Week 52] Evaluation of change from baseline in T-cell responses to AAV5.2 capsid • Systemic biodistribution of ICM-203 [Time Frame: Up to Week 52] Evaluation of presence of ICM-203 in peripheral blood after administration of study drug	Actual Enrollment: Estimated Enrollment: 16 Original Estimated Enrollment: 24 Age: 50 Years to 80 Years (Adult, Older Adult) Sex: All	Study Sponsors: *Same as current* Collaborators: Not Provided	Study Start: March 17, 2022 Primary Completion: March 2024 (Final data collection date for primary outcome measure) Study Completion: June 2024 First Posted: May 6, 2021 Results First Posted: Last Update Posted: September 9, 2022

	NCT Number	Title	Other Names	Status	Conditions	Interventions	Characteristics	Population	Sponsor/Collaborators	Dates
21	NCT05454566	A Study Evaluating the Safety, Tolerability, and Activity of ICM-203 in Subjects With Knee Osteoarthritis. Study Documents:	Title Acronym: Other Ids: ICM 20-1003	Not yet recruiting	Osteoarthritis, Knee	• Genetic: ICM-203 Intra-articular injection • Drug: Placebo (saline solution) Intra-articular injection	Study Type: Interventional Phase: Phase 1 Phase 2 Study Design: Allocation: Randomized Intervention Model: Sequential Assignment Intervention Model Description: • Group 1: ICM-203 6x10e12 vg or Placebo • Group 2: ICM-203 2x10e13 vg or Placebo • Group 3: ICM-203 6x10e13 vg or Placebo Masking: Quadruple (Participant, Care Provider, Investigator, Outcomes Assessor) Primary Purpose: Treatment Primary Outcome Measures: • Treatment-Emergent Adverse Events (TEAEs) [Time Frame: Up to Week 52] Incidence and Severity of Treatment-Emergent Adverse Events following administration of study drug • Knee pain [Time Frame: Up to Week 52] Evaluation of change from baseline in knee pain as measured using a Numerical Rating Scale (NRS) ranging from 0 (no pain) to 10 (worst pain imaginable) • Knee function [Time Frame: Up to Week 52] Evaluation of change from baseline in knee function, pain, and stiffness as measured using the using the Western Ontario and McMaster Universities Osteoarthritis Index (WOMAC) ranging from 0 to 20 (higher scores greater pain) • Articular cartilage grade [Time Frame: Up to Week 52] Evaluation of change from baseline in articular cartilage grade as measured using MRI Osteoarthritis Knee Score (MOAKS) by grading Bone Marrow Lesions; Grade 0= none, grade 1 <33% of subregional volume, grade 2= 33-66% of subregional volume and grade 3 >66% of subregional volume. • Joint space width [Time Frame: Up to Week 52] Evaluation of change from baseline in Joint space width in mm as measured on knee radiograph Secondary Outcome Measures: *Same as current*	Actual Enrollment: Estimated Enrollment: 24 Original Estimated Enrollment: *Same as current* Age: 50 Years to 80 Years (Adult, Older Adult) Sex: All	Study Sponsors: *Same as current* Collaborators: Not Provided	Study Start: December 15, 2022 Primary Completion: June 2024 (Final data collection date for primary outcome measure) Study Completion: December 2024 First Posted: July 12, 2022 Results First Posted: Last Update Posted: September 9, 2022

	NCT Number	Title	Other Names	Status	Conditions	Interventions	Characteristics	Population	Sponsor/Collaborators	Dates
22	NCT01867333	Enzalutamide With or Without Vaccine Therapy for Advanced Prostate Cancer Study Documents:	Title Acronym: Other Ids: 130146 13-C-0146	Active, not recruiting	Prostate Cancer	• Biological: PROSTVAC-F/TRICOM A recombinant fowlpox virus vector vaccine containing the genes for human PSA and three co-stimulatory molecules. • Biological: PROSTVAC-V/TRICOM A recombinant vaccinia virus vector vaccine containing the genes for human PSA and three co-stimulatory molecules. • Biological: Enzalutamide (Xtandi) An androgen receptor inhibitor.	Study Type: Interventional Phase: Phase 2 Study Design: Allocation: Randomized Intervention Model: Parallel Assignment Masking: None (Open Label) Primary Purpose: Treatment Primary Outcome Measures: Increase in time to progression [Time Frame: 4-5 years] Secondary Outcome Measures: • Increase in overall survival [Time Frame: 4-5 years] • Delay in PSA progression [Time Frame: 4-5 years] • Immune response [Time Frame: 4-5 years]	Actual Enrollment: 57 Estimated Enrollment: Original Estimated Enrollment: 76 Age: 18 Years and older (Adult, Older Adult) Sex: Male	Study Sponsors: *Same as current* Collaborators: Not Provided	Study Start: August 12, 2013 Primary Completion: December 1, 2022 (Final data collection date for primary outcome measure) Study Completion: January 1, 2023 First Posted: June 4, 2013 Results First Posted: Last Update Posted: September 9, 2022
23	NCT03767348	Study of RP1 Monotherapy and RP1 in Combination With Nivolumab Study Documents:	Title Acronym: Other Ids: RPL-001-16	Recruiting	• Cancer • Melanoma (Skin) • Mismatch Repair Deficiency • Microsatellite Instability • Non-melanoma Skin Cancer • Cutaneous Melanoma • NSCLC	• Biological: RP1 Genetically modified herpes simplex type 1 virus • Biological: nivolumab anti-PD-1 monoclonal antibody Other Name: Opdivo	Study Type: Interventional Phase: Phase 2 Study Design: Allocation: Non-Randomized Intervention Model: Parallel Assignment Masking: None (Open Label) Primary Purpose: Treatment Primary Outcome Measures: • % subjects with adverse events (AEs) [Time Frame: 26 months] • % subjects with serious adverse events (AEs) [Time Frame: 26 months] • % subjects with dose limiting toxicities (DLTs) [Time Frame: 26 months] • % subjects with overall response (OR) [Time Frame: 26 months] • Maximum tolerated dose (MTD) and recommended Phase 2 dose (RP2D) of RP1 [Time Frame: 20 weeks] Secondary Outcome Measures: • % subjects with biologic activity [Time Frame: 20 weeks] • % subjects with detectable RP1 [Time Frame: 20 weeks] Blood, urine, swabs of injection site, dressing, oral mucosa • % subjects with complete response [Time Frame: 26 months] • median duration of response [Time Frame: 26 months] • median progression free survival [Time Frame: 26 months] • median overall survival [Time Frame: 26 months]	Actual Enrollment: Estimated Enrollment: 300 Original Estimated Enrollment: 168 Age: 18 Years and older (Adult, Older Adult) Sex: All	Study Sponsors: *Same as current* Collaborators: Not Provided	Study Start: September 20, 2017 Primary Completion: November 2024 (Final data collection date for primary outcome measure) Study Completion: November 2024 First Posted: December 6, 2018 Results First Posted: Last Update Posted: September 10, 2022

	NCT Number	Title	Other Names	Status	Conditions	Interventions	Characteristics	Population	Sponsor/Collaborators	Dates
24	NCT04628871	Long Term Follow-up (LTFU) of Subjects Who Received SB-318, SB-913, or SB-FIX Study Documents:	Title Acronym: Other Ids: ST-IVPRP-LT01	Enrolling by invitation	• Hemophilia B • Mucopolysaccharidosis I • Mucopolysaccharidosis II	• Biological: SB-318 No study drug is administered in this study. Subject who received SB-318 in a previous trial will be evaluated in this trial for long-term safety. • Biological: SB-913 No study drug is administered in this study. Subject who received SB-913 in a previous trial will be evaluated in this trial for long-term safety. • Biological: SB-FIX No study drug is administered in this study. Subject who received SB-FIX in a previous trial will be evaluated in this trial for long-term safety.	Study Type: Observational Phase: Study Design: Observational Model: Case-Only Time Perspective: Prospective Primary Outcome Measures: *Same as current* Secondary Outcome Measures: Not Provided	Actual Enrollment: Estimated Enrollment: 13 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: November 3, 2020 Primary Completion: January 1, 2030 (Final data collection date for primary outcome measure) Study Completion: January 1, 2030 First Posted: November 16, 2020 Results First Posted: Last Update Posted: September 13, 2022