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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	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
4	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
5	NCT02858310	E7 TCR T Cells for Human Papillomavirus-Associated Cancers Study Documents:	Title Acronym: Other Ids: 160154 16-C-0154	Recruiting	• Papilloma virus Infections • Cervical Intraepithelial Neoplasia • Carcinoma In Situ • Vulvar Neoplasms • Vulvar Diseases	• Biological: E7 TCR cells T cells genetically engineered with a TCR targeting HPV-16 E7 (E7 TCR) that display specific reactivity against HLA-A2+, HPV-16+ target cells • Drug: Aldesleukin Following cell infusion the patient receives high-dose bolus aldesleukin, which is dosed to individual patient tolerance. Aldesleukin improves the survival of E7 TCR cells after infusion. • Drug: Fludarabine Part of the non-myeloablative lymphocyte-depleting preparative regimen. • Drug: Cyclophosphamide Part of the non-myeloablative lymphocyte-depleting preparative regimen.	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: Determine a safe dose for E7 TCR cells plus aldesleukin with or without pembrolizumab [Time Frame: Phase I, 10 days after treatment] Secondary Outcome Measures: Not Provided	Actual Enrollment: Estimated Enrollment: 180 Original Estimated Enrollment: 40 Age: 18 Years and older (Adult, Older Adult) Sex: All	Study Sponsors: *Same as current* Collaborators: Not Provided	Study Start: January 27, 2017 Primary Completion: December 31, 2024 (Final data collection date for primary outcome measure) Study Completion: January 1, 2026 First Posted: August 8, 2016 Results First Posted: Last Update Posted: September 6, 2022
6	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 12, 2022

	NCT Number	Title	Other Names	Status	Conditions	Interventions	Characteristics	Population	Sponsor/Collaborators	Dates
7	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 6, 2022
8	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

	NCT Number	Title	Other Names	Status	Conditions	Interventions	Characteristics	Population	Sponsor/Collaborators	Dates
9	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 12, 2022
10	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

	NCT Number	Title	Other Names	Status	Conditions	Interventions	Characteristics	Population	Sponsor/Collaborators	Dates
11	NCT05518188	Melpida: Recombinant Adeno-associated Virus (Serotype 9) Encoding a Codon Optimized Human AP4M1 Transgene (hAP4M1opt) Study Documents:	Title Acronym: Other Ids: IND No 028202; Serial No 0000	Not yet recruiting	• Spasticity, Muscle • Microcephaly • Intellectual Deficiency • Growth Retardation • SPG50 • Spastic Paraplegia	Biological: MELPIDA MELPIDA, a recombinant serotype 9 adeno-associated virus (AAV) encoding a codon-optimized human AP4M1 transgene	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: *Same as current*	Actual Enrollment: Estimated Enrollment: 2 Original Estimated Enrollment: *Same as current* Age: 1 Year to 10 Years (Child) Sex: All	Study Sponsors: *Same as current* Collaborators: Cure SPG50	Study Start: October 1, 2022 Primary Completion: October 1, 2028 (Final data collection date for primary outcome measure) Study Completion: October 1, 2030 First Posted: August 26, 2022 Results First Posted: Last Update Posted: September 6, 2022
12	NCT03823131	Optimizing Antitumor Immunity Using Plasmid Electroporation, Pembrolizumab, and Epacadostat Study Documents:	Title Acronym: Other Ids: 172021 NCI-2018-02901 (Registry Identifier: NCI Clinical Trials Reporting Program (CTRP))	Terminated	• Metastatic Head and Neck Squamous Cell Carcinoma • Recurrent Head and Neck Squamous Cell Carcinoma • Unresectable Head and Neck Squamous Cell Carcinoma	• Device: ImmunoPulse Intratumoral Other Names: ○ Electroporation ○ electroporation therapy (EPT) • Drug: Epacadostat Given PO Other Names: ○ INCB 024360 ○ INCB024360 • Drug: Pembrolizumab Given IV Other Names: ○ Keytruda ○ Lambrolizumab ○ MK-3475 ○ SCH 900475 • Biological: CORVax Intratumoral Other Name: DNA-encodable coronaviral vaccine • Drug: Tavokinogene telseplasmid Intratumoral Other Names: ○ Tavo-EP ○ DNA plasmid ○ plasmid IL-12 pUMVC3-hIL-12-NGVL3	Study Type: Interventional Phase: Phase 2 Study Design: Allocation: Non-Randomized Intervention Model: Single Group Assignment Masking: None (Open Label) Primary Purpose: Treatment Primary Outcome Measures: Best overall response rate by Response Evaluation Criteria in Solid Tumors version 1.1 [Time Frame: Up to 36 months] The best overall response(BOR) is the best response recorded from the start of treatment until disease progression/recurrence (taking as reference for progressive disease the smallest measurement recorded since the treatment started). Secondary Outcome Measures: • Incidence of adverse events (AEs) by Common Terminology Criteria for Adverse Events version 4 [Time Frame: Up to 36 months] AEs will be graded and reported descriptively. • Progression free survival (PFS) [Time Frame: From enrollment to progression or last assessment, assessed up to 36 months] PFS is defined as the number of days from enrollment to progression (for subjects who have progression) and the number of days from enrollment to last assessment (for subjects who do not have progression). • Overall survival (OS) [Time Frame: From enrollment to death, or date last known alive, assessed up to 36 months] OS is defined as the number of days from enrollment to death, or from enrollment to date last known alive. • Time to progression [Time Frame: Up to 36 months] Will be summarized using the Kaplan-Meier method.	Actual Enrollment: 14 Estimated Enrollment: Original Estimated Enrollment: 34 Age: 18 Years and older (Adult, Older Adult) Sex: All	Study Sponsors: University of California, San Francisco Collaborators: • Incyte Corporation • OncoSec Medical Incorporated	Study Start: May 2, 2019 Primary Completion: July 31, 2022 (Final data collection date for primary outcome measure) Study Completion: July 31, 2022 First Posted: January 30, 2019 Results First Posted: Last Update Posted: September 6, 2022

	NCT Number	Title	Other Names	Status	Conditions	Interventions	Characteristics	Population	Sponsor/Collaborators	Dates
13	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
14	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

	NCT Number	Title	Other Names	Status	Conditions	Interventions	Characteristics	Population	Sponsor/Collaborators	Dates
15	NCT00895271	Establishing Fibroblast-Derived Cell Lines From Skin Biopsies of Patients With Immunodeficiency or Immunodysregulation Disorders Study Documents:	Title Acronym: Other Ids: 090133 09-I-0133	Enrolling by invitation	• Primary Immunodeficiency • DOCK8 • Virus Susceptibility	Not Provided	Study Type: Observational Phase: Study Design: Observational Model: Cohort Time Perspective: Cross-Sectional Primary Outcome Measures: Not Provided Secondary Outcome Measures: Not Provided	Actual Enrollment: Estimated Enrollment: 200 Original Estimated Enrollment: Age: 2 Years to 85 Years (Child, Adult, Older Adult) Sex: All	Study Sponsors: *Same as current* Collaborators: Not Provided	Study Start: June 10, 2009 Primary Completion: Not Provided Study Completion: Not Provided First Posted: May 8, 2009 Results First Posted: Last Update Posted: September 8, 2022
16	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

	NCT Number	Title	Other Names	Status	Conditions	Interventions	Characteristics	Population	Sponsor/Collaborators	Dates
17	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 12, 2022
18	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 6, 2022

	NCT Number	Title	Other Names	Status	Conditions	Interventions	Characteristics	Population	Sponsor/Collaborators	Dates
19	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
20	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
21	NCT00001823	Evaluation for NCI Surgery Branch Clinical Research Protocols Study Documents:	Title Acronym: Other Ids: 990128 99-C-0128	Recruiting	• Synovial Cell Cancer • Melanoma • Colorectal Cancer • Lung Cancer • Bladder Cancer	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: Estimated Enrollment: 7000 Original Estimated Enrollment: Age: 18 Years and older (Adult, Older Adult) Sex: All	Study Sponsors: *Same as current* Collaborators: Not Provided	Study Start: July 11, 1999 Primary Completion: Not Provided Study Completion: Not Provided First Posted: November 4, 1999 Results First Posted: Last Update Posted: September 6, 2022

	NCT Number	Title	Other Names	Status	Conditions	Interventions	Characteristics	Population	Sponsor/Collaborators	Dates
22	NCT04443907	Study of Safety and Efficacy of Genome-edited Hematopoietic Stem and Progenitor Cells in Sickle Cell Disease (SCD) Study Documents:	Title Acronym: Other Ids: CADPT03A12101 2019-003489-41 (EudraCT Number)	Recruiting	Sickle Cell Disease	• Biological: OTQ923 Single intravenous infusion of OTQ923 cell suspension Other Name: Adult Part A • Biological: OTQ923 Single intravenous infusion of OTQ923, based on review of data from Part A by Health agencies after a formal interim analysis Other Name: Children 2-17 years old - Part B	Study Type: Interventional Phase: Phase 1 Phase 2 Study Design: Allocation: N/A Intervention Model: Single Group Assignment Intervention Model Description: A open label, non-randomized, first-in-patient, phase I/II, proof-of-concept study following subjects for two years after transplantation of either genome-edited autologous HSPC investigational drug product. The study consist of 2 parts - Part A include treatment of adults with OTQ923; Part B include treatment of kids 2-17 years old with either OTQ923 Masking: None (Open Label) Masking Description: The is an open-label study. Primary Purpose: Treatment Primary Outcome Measures: *Same as current* Secondary Outcome Measures: • Durability of hematologic engraftment [Time Frame: 24 months] To assess the durability of hematologic engraftment, HbF expression and edited WBC and bone marrow cells • Number of participants with treatment induced anti-Cas9 humoral and cellular immunogenicity [Time Frame: 24 months] To evaluate presence of pre-existing or treatment induced anti-Cas9 humoral and cellular immunogenicity • Number of participants with event-free survival [Time Frame: 24 months] Overall and event free survival • Evaluation of effect on patient-reported outcomes from baseline and post-HSCT with age appropriate patient reported measures [Time Frame: 24 months] Determine health status following instruments ASCQ-ME emotional impact • Number of participants with change from baseline of annualized VOC rate by 65% [Time Frame: 24 months] Annualized VOC rate • Number of participants with change from baseline of annualized SCD complications (aggregate of VOC, ACS, priapism and stroke) and if relevant, rate of transfusion by 65% [Time Frame: 24 months] Annualized VOC rate • Evaluation of effect on patient-reported outcomes from baseline and post-HSCT with age appropriate patient reported measures [Time Frame: 24 months] Determine health status following instruments PROMIS fatique • Evaluation of effect on patient-reported outcomes from baseline and post-HSCT with age appropriate patient reported measures [Time Frame: 24 months] Determine health status following instruments PROMIS physical functioning • Evaluation of effect on patient-reported outcomes from baseline and post-HSCT with age appropriate patient reported measures [Time Frame: 24 months] Determine health status following instruments ASCQ-ME sleep impact • Evaluation of effect on patient-reported outcomes from	Actual Enrollment: Estimated Enrollment: 20 Original Estimated Enrollment: 30 Age: 2 Years to 40 Years (Child, Adult) Sex: All	Study Sponsors: *Same as current* Collaborators: Not Provided	Study Start: August 26, 2020 Primary Completion: August 19, 2025 (Final data collection date for primary outcome measure) Study Completion: August 19, 2025 First Posted: June 23, 2020 Results First Posted: Last Update Posted: September 6, 2022

	NCT Number	Title	Other Names	Status	Conditions	Interventions	Characteristics	Population	Sponsor/Collaborators	Dates
23	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
24	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 12, 2022