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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	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

	NCT Number	Title	Other Names	Status	Conditions	Interventions	Characteristics	Population	Sponsor/Collaborators	Dates
7	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
8	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 9, 2022

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

	NCT Number	Title	Other Names	Status	Conditions	Interventions	Characteristics	Population	Sponsor/Collaborators	Dates
11	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
12	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
13	NCT03602612	T Cells Expressing a Novel Fully-Human Anti-BCMA CAR for Treating Multiple Myeloma Study Documents:	Title Acronym: Other Ids: 180125 18-C-0125	Active, not recruiting	• Myeloma-Multiple • Myeloma, Plasma-Cell	• Drug: Cyclophosphamide 300 mg/m^2 IV over 30 minutes on days -5, -4, and -3 • Drug: Fludarabine 30 mg/m^2 IV infusion over 30 minutes administered immediately following the cyclophosphamide on day -5, -4, -3 • Biological: Anti-BCMA CAR T cells 0.75x10^6 - 12.0X10^6 CAR+ T cells per kg of recipient bodyweight one time dose on day 0	Study Type: Interventional Phase: Phase 1 Study Design: Allocation: Non-Randomized Intervention Model: Sequential Assignment Masking: None (Open Label) Primary Purpose: Treatment Primary Outcome Measures: *Same as current* Secondary Outcome Measures: Not Provided	Actual Enrollment: 35 Estimated Enrollment: Original Estimated Enrollment: 42 Age: 18 Years to 73 Years (Adult, Older Adult) Sex: All	Study Sponsors: *Same as current* Collaborators: Not Provided	Study Start: September 14, 2018 Primary Completion: January 1, 2023 (Final data collection date for primary outcome measure) Study Completion: January 1, 2024 First Posted: July 27, 2018 Results First Posted: Last Update Posted: September 9, 2022
14	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
15	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
16	NCT02830724	Administering Peripheral Blood Lymphocytes Transduced With a CD70-Binding Chimeric Antigen Receptor to People With CD70 Expressing Cancers Study Documents:	Title Acronym: Other Ids: 160131 16-C-0131	Recruiting	• Pancreatic Cancer • Renal Cell Cancer • Breast Cancer • Melanoma • Ovarian Cancer	• Drug: Cyclophosphamide For Phase I, Days -7 and -6: Dose Level 1: 15 mg/kg/day x 2 days IV Dose Level 2: 15 mg/kg/day x 2 days IV Dose Level 3: 15 mg/kg/day x 2 days IV Dose Level 4: 15 mg/kg/day x 2 days IV Dose Level 5: 30 mg/kg/day x 2 days IV Dose Level 6: 60 mg/kg/day x 2 days IV For Phase II, Days -7 and -6: 60 mg/kg/day x 2 days IV • Drug: Fludarabine For Phase I, Days -7 to -5: Dose Level 1: 25 mg/m(2)/day x 3 days IVPB Dose Level 2: 25 mg/m(2)/day x 3 days IVPB Dose Level 3: 25 mg/m(2)/day x 3 days IVPB Dose Level 4: 25 mg/m(2)/day x 3 days IVPB Dose Level 5: 25 mg/m(2)/day x 5 days IVPB Dose Level 6: 25 mg/m(2)/day x 5 days IVPB For Phase II, Days -7 to -3: 25 mg/m(2)/day x 5 days IVPB • Drug: Aldesleukin Aldeskeukin 720,000 IU/kg IV (based on total body weight) over 15 minutes approximately every 8 hours beginning within 24 hours of cell infusion and continuing for up to 3 days (maximum 9 doses). • Biological: Anti-hCD70 CAR transduced PBL Day 0: Cells will be infused intravenously on the Patient Care Unit over 20-30 minutes (2-5 days after the last dose of fludarabine).	Study Type: Interventional Phase: Phase 1 Phase 2 Study Design: Allocation: Non-Randomized Intervention Model: Sequential Assignment Masking: None (Open Label) Primary Purpose: Treatment Primary Outcome Measures: To determine the safety of administering PBL transduced with this anti-CD70 CAR in concert with preparative lymphodepletion and high dose interleukin-2 (IL-2; aldesleukin) and to mediate regression. [Time Frame: Approximately 5 years] Secondary Outcome Measures: • Determine the in vivo survival of anti-hCD70 CAR transduced cells [Time Frame: Approximately 5 years] • Determine the toxicity of this treatment regimen [Time Frame: Approximately 5 years]	Actual Enrollment: Estimated Enrollment: 124 Original Estimated Enrollment: 113 Age: 18 Years to 70 Years (Adult, Older Adult) Sex: All	Study Sponsors: *Same as current* Collaborators: Not Provided	Study Start: April 6, 2017 Primary Completion: January 1, 2027 (Final data collection date for primary outcome measure) Study Completion: January 1, 2028 First Posted: July 13, 2016 Results First Posted: Last Update Posted: September 6, 2022

	NCT Number	Title	Other Names	Status	Conditions	Interventions	Characteristics	Population	Sponsor/Collaborators	Dates
17	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
18	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
19	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
20	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
21	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

	NCT Number	Title	Other Names	Status	Conditions	Interventions	Characteristics	Population	Sponsor/Collaborators	Dates
22	NCT03435796	Long-Term Follow-up Protocol for Participants Treated With Gene-Modified T Cells Study Documents:	Title Acronym: Other Ids: GC-LTFU-001 U1111-1206-8250 (Registry Identifier: WHO) 2017-001465-24 (EudraCT Number)	Recruiting	Neoplasms	Genetic: Gene-modified (GM) T cell therapy No investigational product will be administered	Study Type: Interventional Phase: Phase 2 Phase 3 Study Design: Allocation: N/A Intervention Model: Single Group Assignment Masking: None (Open Label) Primary Purpose: Other Primary Outcome Measures: • Adverse Events (AEs) [Time Frame: Up to 15 years from last GM T cell infusion] Incidence of delayed Adverse Events suspected to be related to prior gene-modified (GM) T cell therapy • Tumor Response Status [Time Frame: At month 12 from last GM T cells infusion then yearly until date of disease relapse or progression, assessed up to year 15] Number of subjects who continue to be responders, who have progressed, and who have relapsed will be reported. When reporting progression/relapse the appropriate date will also be reported. • Disease Progression [Time Frame: Up to 15 years from last GM T cells infusion] Number of subjects who continue to be responders, who have progressed, and who have relapsed will be reported. When reporting progression/relapse the appropriate date will also be reported. • Disease Relapse [Time Frame: Up to 15 years from last GM T cells infusion] Number of subjects who continue to be responders, who have progressed, and who have relapsed will be reported. When reporting progression/relapse the appropriate date will also be reported. • Overall Survival [Time Frame: Up to 15 years from last GM T cells infusion] Overall survival is defined as the time from the first dose of investigational product or from the randomization date to the date of death or the date the subject is last known to be alive. • Health-related quality of life (HRQoL) [Time Frame: Up to approximately 5 years] Health-related quality of life (HRQoL) analyses will be performed by combining data collected in this protocol with the data from each respective parent study as appropriate per parent study specified analysis plan, and per LTFU study plan after the parent study is closed out. • Height of pediatric subjects treated with GM T cells [Time Frame: At month 12 from last GM T cells infusion then yearly until subject reaches Stage 5 per Tanner staging criteria or for 15 years from last GM T cell infusion, whichever occurs later] Height (inches or centimeters) will be collected for all pediatric subjects and descriptively summarized • Weight of pediatric subjects treated with GM T cells [Time Frame: At month 12 from last GM T cells infusion then yearly until subject reaches Stage 5 per Tanner staging criteria or for 15 years from last GM T cell infusion, whichever occurs later] Weight (pounds or kilograms) will be collected for all pediatric subjects and descriptively summarized • Sexual maturation of pediatric subjects treated with GM T cells [Time Frame: At month 12 from last GM T cells infusion then yearly until subject reaches Stage 5 per Tanner staging criteria or for 15 years from last GM T cell infusion, whichever occurs later]	Actual Enrollment: Estimated Enrollment: 191 Original Estimated Enrollment: 200 Age: Child, Adult, Older Adult Sex: All	Study Sponsors: *Same as current* Collaborators: Not Provided	Study Start: June 19, 2018 Primary Completion: November 30, 2036 (Final data collection date for primary outcome measure) Study Completion: November 30, 2036 First Posted: February 19, 2018 Results First Posted: Last Update Posted: September 7, 2022

	NCT Number	Title	Other Names	Status	Conditions	Interventions	Characteristics	Population	Sponsor/Collaborators	Dates
23	NCT05158296	Study to Evaluate the Efficacy Safety and Tolerability of Ultevursen in Subjects With RP Due to Mutations in Exon 13 of the USH2A Gene (Sirius) Study Documents:	Title Acronym: Other Ids: PQ-421a-003 2021-002729-74 (EudraCT Number)	Active, not recruiting	• Retinitis Pigmentosa • Usher Syndrome Type 2 • Deaf Blind • Retinal Disease • Eye Diseases • Eye Diseases, Hereditary • Eye Disorders Congenital • Vision Disorders	• Drug: Ultevursen RNA antisense oligonucleotide for intravitreal injection Other Name: RNA antisense oligonucleotide for intravitreal injection • Other: Sham-procedure Sham-procedure (no experimental drug administered)	Study Type: Interventional Phase: Phase 2 Phase 3 Study Design: Allocation: Randomized Intervention Model: Parallel Assignment Intervention Model Description: Double-masked, randomized, controlled, multiple-dose study. Subjects will be randomized to one of three treatment groups: 1. Group 1: Ultevursen 180/60 µg (180 µg loading dose administered on Day 1, 60 µg maintenance dose administered at Month 3 and every 6 months thereafter) 2. Group 2: Ultevursen 60/60 µg (60 µg loading dose administered on Day 1, 60 µg maintenance dose administered at Month 3 and every 6 months thereafter; n = 27) 3. Group 3: Sham-procedure (administered on Day 1, Month 3 and every 6 months thereafter; n = 27) Masking: Quadruple (Participant, Care Provider, Investigator, Outcomes Assessor) Masking Description: Subject, site staff (study coordinator, imaging technician, etc) and Investigator will be completely masked. Physician performing IVT and post-IVT monitoring will know if subject is receiving sham or treatment, but will be masked to the dose level. Pharmacist is the only site staff that will be completely unmasked. Primary Purpose: Treatment Primary Outcome Measures: Change from baseline in BCVA [Time Frame: 18 months of treatment versus sham-procedure] Change from baseline in best corrected visual acuity(BCVA) based on the Early Treatment Diabetic Retinopathy Study (ETDRS) chart Secondary Outcome Measures: • Proportion of patients who maintain vision defined by BCVA loss less than 15 Letters (ETDRS) [Time Frame: 27 months] Proportion of patients who maintain vision defined by BCVA loss less than 15 Letters (ETDRS) • Change from baseline in other measures of BCVA [Time Frame: 27 months] Change from baseline in other measures of BCVA • Change from baseline in spectral domain optical coherence tomography (SD-OCT) [Time Frame: 27 months] Change from baseline in spectral domain optical coherence tomography (SD-OCT) • Change from baseline in Low Luminance Visual Acuity (LLVA) [Time Frame: 27 months] Change from baseline in Low Luminance Visual Acuity (LLVA) • Change from baseline in Microperimetry [Time Frame: 27 months] Change from baseline in Microperimetry • Change from baseline in Static perimetry [Time Frame: 27 months] Change from baseline in Static perimetry • Change from baseline in Full-field Stimulus Threshold (FST) [Time Frame: 27 months] Change from baseline in Full-field Stimulus Threshold (FST)	Actual Enrollment: Estimated Enrollment: 81 Original Estimated Enrollment: *Same as current* Age: 12 Years and older (Child, Adult, Older Adult) Sex: All	Study Sponsors: *Same as current* Collaborators: Not Provided	Study Start: December 8, 2021 Primary Completion: December 2024 (Final data collection date for primary outcome measure) Study Completion: December 2024 First Posted: December 15, 2021 Results First Posted: Last Update Posted: September 8, 2022

	NCT Number	Title	Other Names	Status	Conditions	Interventions	Characteristics	Population	Sponsor/Collaborators	Dates
24	NCT05050084	Two Studies for Patients With Unfavorable Intermediate Risk Prostate Cancer Testing Less Intense Treatment for Patients With a Low Gene Risk Score and Testing a More Intense Treatment for Patients With a Higher Gene Risk Score Study Documents:	Title Acronym: Other Ids: NRG-GU010 NCI-2021-08760 (Registry Identifier: CTRP (Clinical Trial Reporting Program)) NRG-GU010 (Other Identifier: NRG Oncology) NRG-GU010 (Other Identifier: CTEP) U10CA180868 (U.S. NIH Grant/Contract)	Recruiting	Prostate Adenocarcinoma	• Drug: Bicalutamide Anti-androgen Other Names: ○ Casodex ○ Cosudex ○ ICI 176,334 ○ ICI 176334 • Drug: Buserelin GnRH agonist Other Names: ○ 6-[O-(1,1-Dimethylethyl)-D-serine]-9-(N-ethyl-L-prolinamide)-10-deglycinamide-luteinizing Hormone-releasing Factor (Pig) ○ BSRL ○ Busereline ○ Etilamide ○ HOE 766 ○ ICI 123215 ○ S74-6766 • Drug: Darolutamide Anti-androgen Other Names: ○ Antiandrogen ODM-201 ○ BAY 1841788 ○ BAY-1841788 ○ BAY1841788 ○ Nubeqa ○ ODM 201 ○ ODM-201 • Drug: Degarelix GnRH antagonist Other Names: ○ FE200486 ○ Firmagon • Drug: Flutamide Anti-androgen Other Names: ○ 4'-Nitro-3'-trifluoromethylisobutyranilide ○ Apimid ○ Cebatrol ○ Chimax ○ Cytomid ○ Drogenil ○ Euflex ○ Eulexine ○ Flucinom ○ Flucinome ○ Flugerel ○ Fluken ○ Flulem ○ FLUT ○ Fluta-Gry ○ Flutabene ○ Flutacan ○ Flutamex ○ Flutamin ○ Flutan ○ Flutaplex ○ Fugerel ○ Grisetin ○ Niftolide ○ Oncosal ○ Profamid ○ Propanamide, 2-Methyl-N-(4-nitro-3-(trifluoromethyl)phenyl)- ○ Prostacur ○ Prostadirex	Study Type: Interventional Phase: Phase 3 Study Design: Allocation: Randomized Intervention Model: Parallel Assignment Masking: None (Open Label) Primary Purpose: Treatment Primary Outcome Measures: • Distant metastasis (DM) (De-intensification study) [Time Frame: From randomization to the detection of distant metastasis by conventional imaging, assessed up to 5 years] • Metastasis-free survival (MFS) (Intensification study) [Time Frame: From randomization until the occurrence of distant metastasis by conventional imaging or death from any cause, assessed up to 5 years] MFS will be estimated by the Kaplan-Meier (1958) method and compared between the two treatment arms using a stratified log-rank test (stratified by the randomization stratification factors) at one-sided alpha level of 0.025. Secondary Outcome Measures: • Overall survival [Time Frame: From randomization to death from any cause, assessed up to 5 years] Will be estimated by the Kaplan-Meier method and compared between treatments arms by stratified log-rank test. Cox regression models will also be fit, adjusted for the stratification factors, to estimate hazard ratios, together with 95% confidence intervals. • Time to prostate specific antigen (PSA) failure [Time Frame: Up to 5 years] Defined as PSA > 2 ng/ml above the nadir post randomization. Will be analyzed using competing-risk methods (Gooley 1999) where, in each case, death prior to occurrence of the event in question will be a competing risk. • MFS (De-intensification study) [Time Frame: From randomization until the occurrence of distant metastasis by conventional imaging or death from any cause, assessed up to 5 years] Will be estimated by the Kaplan-Meier method and compared between treatments arms by stratified log-rank test. Cox regression models will also be fit, adjusted for the stratification factors, to estimate hazard ratios, together with 95% confidence intervals. • MFS including positron emission tomography (PET) imaging [Time Frame: From randomization until the occurrence of distant metastasis by conventional and/or molecular imaging or death from any cause, assessed up to 5 years] Will be estimated by the Kaplan-Meier method and compared between treatments arms by stratified log-rank test. • Locoregional failure (LRF) [Time Frame: From randomization until local or regional recurrence based upon conventional imaging or biopsy, assessed up to 5 years] Will compare cumulative incidence between arms. • DM including PET imaging [Time Frame: From randomization to the detection of distant metastasis by conventional and/or molecular imaging, assessed up to 5 years] Will be analyzed using competing-risk methods (Gooley 1999) where, in each case, death prior to occurrence of	Actual Enrollment: Estimated Enrollment: 2050 Original Estimated Enrollment: *Same as current* Age: 18 Years and older (Adult, Older Adult) Sex: Male	Study Sponsors: *Same as current* Collaborators: National Cancer Institute (NCI)	Study Start: November 3, 2021 Primary Completion: April 30, 2032 (Final data collection date for primary outcome measure) Study Completion: April 30, 2037 First Posted: September 20, 2021 Results First Posted: Last Update Posted: September 8, 2022

	NCT Number	Title	Other Names	Status	Conditions	Interventions	Characteristics	Population	Sponsor/Collaborators	Dates
25	NCT05529862	Trans-RosaLEE Study: a Biomarker-directed, Translational Study Study Documents:	Title Acronym: Other Ids: TransRosaLEE-IPC 2021-075	Not yet recruiting	Advanced or Metastatic Breast Cancer (BC)	• Genetic: Pre-treatment biopsy Pre-treatment fragments will be collected during the biopsy visit organised as part of routine medical practice, prior to the start of treatment with ribociclib + ET • Genetic: Post treatment biopsy Post-treatment fragments will be collected during a biopsy visit specifically planned for Trans-RosaLEE study. • Genetic: Pre treatment blood sampling Sampling of 4 EDTA Tubes (4ml) and 2 Streck tubes (10ml) • Genetic: Post treatment blood sampling Sampling of 2 Streck tubes (10ml)	Study Type: Interventional Phase: Not Applicable Study Design: Allocation: N/A Intervention Model: Single Group Assignment Masking: None (Open Label) Primary Purpose: Basic Science Primary Outcome Measures: *Same as current* Secondary Outcome Measures: *Same as current*	Actual Enrollment: Estimated Enrollment: 241 Original Estimated Enrollment: *Same as current* Age: 18 Years and older (Adult, Older Adult) Sex: Female	Study Sponsors: *Same as current* Collaborators: Novartis Pharmaceuticals	Study Start: October 1, 2022 Primary Completion: April 1, 2026 (Final data collection date for primary outcome measure) Study Completion: October 1, 2027 First Posted: September 7, 2022 Results First Posted: Last Update Posted: September 7, 2022