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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	NCT03861273	A Study to Evaluate the Efficacy and Safety of Factor IX Gene Therapy With PF-06838435 in Adult Males With Moderately Severe to Severe Hemophilia B Study Documents:	Title Acronym: Other Ids: C0371002 2018-003086-33 (EudraCT Number)	Active, not recruiting	Hemophilia B	Biological: PF-06838435/ fidanacogene elaparvovec Gene Therapy	Study Type: Interventional Phase: Phase 3 Study Design: Allocation: Non-Randomized Intervention Model: Single Group Assignment Masking: None (Open Label) Primary Purpose: Treatment Primary Outcome Measures: • Annualized bleeding rate (ABR) [Time Frame: First 12 months post PF 06838435 infusion] • Vector derived FIX:C level [Time Frame: Week 12 to 12 months post PF 06838435 infusion] Secondary Outcome Measures: • Annualized infusion rate (AIR) of exogenous Factor IX Activity [Time Frame: First 12 months post study drug infusion] • Annualized Factor IX Activity consumption [Time Frame: 12 months post study drug infusion] • Annualized number of bleeding events of specific type: spontaneous and traumatic, and untreated [Time Frame: 12 months post study drug infusion] • Frequency of target joint bleeds [Time Frame: 12 months post study drug infusion] • Percentage of the participants without bleeds [Time Frame: 12 months post study drug infusion] • Change in joint health as measured by the Hemophilia Joint Health Score (HJHS) instrument [Time Frame: 12 months post study drug infusion] • Patient Reported Outcome (PRO) instrument - Hemophilia Quality of Life (Haem A QoL) [Time Frame: 12 months post study drug infusion] • Patient Reported Outcome (PRO) instrument - Hemophilia Activities List (HAL) [Time Frame: 12 months post study drug infusion] • Patient Reported Outcome (PRO) instrument - Patient Global Impression of Change-Hemophilia (PGIC-H) [Time Frame: 12 months post study drug infusion] • Annualized Bleeding Rate [Time Frame: Annually for 6 years] • Vector derived Factor IX activity (FIX:C) level at steady state [Time Frame: Annually for 6 years] • Annualized infusion rate (AIR) of exogenous Factor IX [Time Frame: Annually for 6 years] • Annualized Factor IX consumption [Time Frame: Annually for 6 years] • Annualized number of bleeding events of specific type: spontaneous and traumatic, and untreated [Time Frame: Annually for 6 years] • Frequency of target joint bleeds [Time Frame: Annually for 6 years] • Patient Reported Outcome (PRO) instrument - Hemophilia Quality of Life (Haem A QoL) [Time Frame: Annually for 6 years] • Patient Reported Outcome (PRO) instrument - Hemophilia Activities List (HAL) [Time Frame: Annually for 6 years] • Patient Reported Outcome (PRO) instrument - Patient Global Impression of Change - Hemophilia (PGIC-H) [Time Frame: Annually for 6 years] • Incidence and severity of all adverse events collected during the study [Time Frame: For the duration of 6	Actual Enrollment: 45 Estimated Enrollment: Original Estimated Enrollment: 55 Age: 18 Years to 65 Years (Adult, Older Adult) Sex: Male	Study Sponsors: *Same as current* Collaborators: Not Provided	Study Start: July 29, 2019 Primary Completion: November 21, 2022 (Final data collection date for primary outcome measure) Study Completion: March 11, 2030 First Posted: March 4, 2019 Results First Posted: Last Update Posted: September 14, 2022

	NCT Number	Title	Other Names	Status	Conditions	Interventions	Characteristics	Population	Sponsor/Collaborators	Dates
4	NCT04798235	First-in-Human Study of TSHA-101 Gene Therapy for Treatment of Infantile Onset GM2 Gangliosidosis Study Documents:	Title Acronym: Other Ids: TSHA-101-IST-001	Active, not recruiting	Infantile GM2 Gangliosidosis (Disorder)	Biological: TSHA-101 AAV9 viral vector containing HEXA and HEXB genes to be administered via Intrathecal injection	Study Type: Interventional Phase: Phase 1 Phase 2 Study Design: Allocation: N/A Intervention Model: Single Group Assignment Masking: None (Open Label) Primary Purpose: Treatment Primary Outcome Measures: *Same as current* Secondary Outcome Measures: • Safety and tolerability: Viral shedding analysis [Time Frame: 1 year] Positive presence of viral DNA from biological fluids (whole blood, urine, saliva, and stool) • Assessment of Immunogenicity: Biomarkers in serum milestones [Time Frame: 1 year] Summary of neutralizing antibodies (NAbs) titers for adeno-associated virus, serotype 9 (AAV9) and Hex A • Assessment of Immunogenicity: Biomarkers in serum [Time Frame: 1 year] Summary of total antibodies (TAbs) titers for AAV9 and Hex A • Assessment of Immunogenicity: Biomarkers in peripheral blood mononuclear cells (PBMCs [Time Frame: 5 years] Summary of PBMCs for enzyme-linked immune absorbent spot (ELISpot) assays for cytokine secretion against AAV9 and Hex A • Overall Survival [Time Frame: treatment to death from any cause, up to 5 years] Estimated using the Kaplan-Meier method • Hex A Enzyme Activity: Cerebrospinal fluid (CSF) and serum [Time Frame: 1 year] Change from baseline • Head Control: Number of events for abnormal head control [Time Frame: 1 year] change from Baseline • Change from Baseline in motor function: Children's Hospital of Philadelphia Infant Test of Neuromuscular Disorders (CHOP-INTEND) [Time Frame: 1 year] The test consists of 16 items (body parts), where each item is tested for both sides of the body, left and right. The best score is taken for each item (with a maximum score of 4), and the scores are summed over all 16 items with a possible total CHOP-INTEND score of 64. • Change from Baseline in Motor Function: Modified Ashworth Scale [Time Frame: 1 year] change from Baseline. Increase or decrease of muscle tone will be measured by the Modified Ashworth Scale. Frequency counts and percentages will be presented by score (0, 1, 1+, 2, 3, and 4), muscle, side, and visit for the safety population. Flexion and extension of the knee and elbow will be measured on both sides, along with hip adduction and abduction on both sides of the body. • Clinical Efficacy Assessment: Progression of Hypotonia [Time Frame: 1 year] Assessed through neurological examinations as present or absent. Baseline to each post-Baseline visit • Clinical Efficacy Assessment: Dysphagia [Time Frame: From onset up to 3 years, if present] Assessment of the dysphagia events- assessed as present	Actual Enrollment: 3 Estimated Enrollment: Original Estimated Enrollment: 6 Age: up to 15 Months (Child) Sex: All	Study Sponsors: *Same as current* Collaborators: • Taysha Gene Therapies, Inc. • GlycoNet	Study Start: March 12, 2021 Primary Completion: March 12, 2023 (Final data collection date for primary outcome measure) Study Completion: March 12, 2027 First Posted: March 15, 2021 Results First Posted: Last Update Posted: September 14, 2022

	NCT Number	Title	Other Names	Status	Conditions	Interventions	Characteristics	Population	Sponsor/Collaborators	Dates
5	NCT01976091	A Gene Transfer Therapy Study to Evaluate the Safety of SRP-9004 (Patidistrogene Bexoparvovec) in Participants With Limb-Girdle Muscular Dystrophy, Type 2D (LGMD2D) Study Documents:	Title Acronym: Other Ids: 9004-101 5U01AR060911 (U.S. NIH Grant/Contract)	Completed	Limb-Girdle Muscular Dystrophy, Type 2D	Genetic: SRP-9004 Isolated Limb Infusion (ILI) Other Name: patidistrogene bexoparvovec	Study Type: Interventional Phase: Phase 1 Phase 2 Study Design: Allocation: Non-Randomized Intervention Model: Sequential Assignment Masking: None (Open Label) Primary Purpose: Treatment Primary Outcome Measures: Safety with fewer than 2 grade 3 adverse events [Time Frame: 1 year from start] Safety with fewer than 2 grade 3 adverse events Secondary Outcome Measures: Efficacy outcome measure 6MWT [Time Frame: 2 years] 6 minute walk test (6MWT)-(primary variable to measure efficacy) Efficacy would be a significant improvement in distance walked in the 6 minute walk test.	Actual Enrollment: 6 Estimated Enrollment: Original Estimated Enrollment: *Same as current* Age: 7 Years and older (Child, Adult, Older Adult) Sex: All	Study Sponsors: Jerry R. Mendell Collaborators: Nationwide Children's Hospital	Study Start: February 1, 2015 Primary Completion: March 14, 2019 (Final data collection date for primary outcome measure) Study Completion: March 14, 2019 First Posted: April 1, 2022 Results First Posted: April 1, 2022 Last Update Posted: September 13, 2022
6	NCT03588299	Study to Test the Safety and How Well Patients With Severe Hemophilia A Respond to Treatment With BAY 2599023 (DTX 201), a Drug Therapy That Delivers a Healthy Version of the Defective Factor VIII Gene Into the Nucleus of Liver Cells Using an Altered, Non-infectious Virus (AAV) as a "Shuttle". Study Documents:	Title Acronym: Other Ids: 19429 2017-000806-39 (EudraCT Number)	Active, not recruiting	Hemophilia A	Drug: BAY2599023 (DTX201) Single escalating doses with 4 dose steps; Single intravenous (IV) administration.	Study Type: Interventional Phase: Phase 1 Phase 2 Study Design: Allocation: N/A Intervention Model: Single Group Assignment Masking: None (Open Label) Primary Purpose: Treatment Primary Outcome Measures: Number of patients with adverse events (AEs), treatment-emergent adverse events (TEAEs), serious adverse events (SAEs) and AEs/SAEs of special interest [Time Frame: Up to 52 weeks] Secondary Outcome Measures: Change of FVIII activity from baseline throughout the study [Time Frame: Up to 5 years] FVIII activity will be determined using both a one-stage assay and chromogenic assay.	Actual Enrollment: 11 Estimated Enrollment: Original Estimated Enrollment: 18 Age: 18 Years and older (Adult, Older Adult) Sex: Male	Study Sponsors: *Same as current* Collaborators: Ultragenix pharmaceutical	Study Start: November 7, 2018 Primary Completion: November 3, 2026 (Final data collection date for primary outcome measure) Study Completion: November 30, 2026 First Posted: July 17, 2018 Results First Posted: Last Update Posted: September 14, 2022

	NCT Number	Title	Other Names	Status	Conditions	Interventions	Characteristics	Population	Sponsor/Collaborators	Dates
7	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

	NCT Number	Title	Other Names	Status	Conditions	Interventions	Characteristics	Population	Sponsor/Collaborators	Dates
8	NCT04055090	Extension of Phase 3 Gene Therapy for Painful Diabetic Neuropathy Study Documents:	Title Acronym: Other Ids: VMDN-003b	Completed	• Painful Diabetic Neuropathy • Diabetic Neuropathy, Painful	• Genetic: Long-Term Follow-Up of Patients who Received Engensis (VM202) No study drug is administered in this study. Patients who received Engensis (VM202) in a previous trial will be evaluated in this trial for long-term safety and efficacy. • Drug: Long-Term Follow-Up of Patients who Received Placebo No study drug is administered in this study. Patients who received Placebo in a previous trial will be evaluated in this trial for long-term safety and efficacy.	Study Type: Interventional Phase: Phase 3 Study Design: Allocation: Randomized Intervention Model: Parallel Assignment Intervention Model Description: Long term, prospective, non-interventional, safety extension study of phase 3 trial. Double blind, randomized, placebo-controlled, multicenter study/ Masking: Quadruple (Participant, Care Provider, Investigator, Outcomes Assessor) Masking Description: Double-blind Primary Purpose: Treatment Primary Outcome Measures: the difference in long-term safety [Time Frame: Baseline through Day 365 follow up] defined as occurrence of adverse events - observed between subjects receiving VM202 versus subjects receiving placebo in the VMDN-003 study Secondary Outcome Measures: • The change in the average 24-hour pain score from baseline to the Day 365 follow-up [Time Frame: baseline to the Day 365 follow-up] The change in the average 24-hour pain score from baseline to the Day 365 follow-up from the Daily Pain and Sleep Interference Diary • The change in the average 24-hour pain score from Day 270 to the Day 365 follow-up [Time Frame: Day 270 to the Day 365 follow-up] The change in the average 24-hour pain score from Day 270 to the Day 365 follow-up from the Daily Pain and Sleep Interference Diary; • Patient's Global Impression of Change (PGIC) at the Day 365 follow-up [Time Frame: At the Day 365 follow-up] The patient's global impression of change	Actual Enrollment: 101 Estimated Enrollment: Original Estimated Enrollment: 120 Age: 18 Years to 75 Years (Adult, Older Adult) Sex: All	Study Sponsors: *Same as current* Collaborators: Not Provided	Study Start: February 4, 2019 Primary Completion: July 24, 2019 (Final data collection date for primary outcome measure) Study Completion: July 24, 2019 First Posted: August 13, 2019 Results First Posted: Last Update Posted: September 14, 2022

	NCT Number	Title	Other Names	Status	Conditions	Interventions	Characteristics	Population	Sponsor/Collaborators	Dates
9	NCT04281485	Study to Evaluate the Safety and Efficacy of PF-06939926 for the Treatment of Duchenne Muscular Dystrophy Study Documents:	Title Acronym: Other Ids: C3391003 2019-002921-31 (EudraCT Number)	Recruiting	Duchenne Muscular Dystrophy	• Genetic: PF-06939926 PF-06939926 will be administered as a single IV infusion at Year 1 for Cohort 1. • Other: Placebo Placebo will be administered as a single IV infusion at Year 1 for Cohort 2. • Other: Placebo Placebo will be administered as a single IV infusion at Year 2 for Cohort 1. • Genetic: PF-06939926 PF-06939926 will be administered as a single IV infusion at Year 2 for Cohort 2	Study Type: Interventional Phase: Phase 3 Study Design: Allocation: Randomized Intervention Model: Parallel Assignment Intervention Model Description: Parallel up to the measurement of the primary outcome at Week 52. At the beginning of study Year 2 participants who were originally assigned to placebo will have the opportunity to receive PF-06939926. All participants will be followed for 5 years following treatment with PF-06939926. Masking: Quadruple (Participant, Care Provider, Investigator, Outcomes Assessor) Masking Description: The study will be quadruple blind. Primary Purpose: Treatment Primary Outcome Measures: *Same as current* Secondary Outcome Measures: *Same as current*	Actual Enrollment: Estimated Enrollment: 99 Original Estimated Enrollment: *Same as current* Age: 4 Years to 7 Years (Child) Sex: Male	Study Sponsors: *Same as current* Collaborators: Not Provided	Study Start: November 5, 2020 Primary Completion: January 30, 2024 (Final data collection date for primary outcome measure) Study Completion: January 29, 2029 First Posted: February 24, 2020 Results First Posted: Last Update Posted: September 14, 2022
10	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

	NCT Number	Title	Other Names	Status	Conditions	Interventions	Characteristics	Population	Sponsor/Collaborators	Dates
11	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
12	NCT01621581	AAV2-GDNF for Advanced Parkinson s Disease Study Documents:	Title Acronym: Other Ids: 120137 12-N-0137	Completed	Parkinson's Disease	Genetic: Convection enhanced delivery/AAV2-GDNF Adeno-Associated Virus Encoding Glial Cell Line-Derived Neurotrophic Factor (AAV2-GDNF) Administered via Bilateral Stereotactic Convection-Enhanced Delivery	Study Type: Interventional Phase: Phase 1 Study Design: Allocation: Non-Randomized Intervention Model: Single Group Assignment Masking: None (Open Label) Primary Purpose: Treatment Primary Outcome Measures: Assess the safety and tolerability of 4 different dose levels of AAV2-GDNF Secondary Outcome Measures: Obtain preliminary data regarding the potential for clinical responses of the 4 dose levels tested by assessing the magnitude and variability of any treatment effects (via clinical, laboratory and neuroimaging studies).	Actual Enrollment: 25 Estimated Enrollment: Original Estimated Enrollment: 28 Age: 18 Years and older (Adult, Older Adult) Sex: All	Study Sponsors: *Same as current* Collaborators: Not Provided	Study Start: March 13, 2013 Primary Completion: February 4, 2022 (Final data collection date for primary outcome measure) Study Completion: February 4, 2022 First Posted: June 18, 2012 Results First Posted: Last Update Posted: September 9, 2022

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

	NCT Number	Title	Other Names	Status	Conditions	Interventions	Characteristics	Population	Sponsor/Collaborators	Dates
15	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 10, 2022
16	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
17	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
18	NCT03602612	T Cells Expressing a Novel Fully-Human Anti-BCMA CAR for Treating Multiple Myeloma Study Documents:	Title Acronym: Other Ids: 180125 18-C-0125	Active, not recruiting	• Myeloma-Multiple • Myeloma, Plasma-Cell	• Drug: Cyclophosphamide 300 mg/m^2 IV over 30 minutes on days -5, -4, and -3 • Drug: Fludarabine 30 mg/m^2 IV infusion over 30 minutes administered immediately following the cyclophosphamide on day -5, -4, -3 • Biological: Anti-BCMA CAR T cells 0.75x10^6 - 12.0X10^6 CAR+ T cells per kg of recipient bodyweight one time dose on day 0	Study Type: Interventional Phase: Phase 1 Study Design: Allocation: Non-Randomized Intervention Model: Sequential Assignment Masking: None (Open Label) Primary Purpose: Treatment Primary Outcome Measures: *Same as current* Secondary Outcome Measures: Not Provided	Actual Enrollment: 35 Estimated Enrollment: Original Estimated Enrollment: 42 Age: 18 Years to 73 Years (Adult, Older Adult) Sex: All	Study Sponsors: *Same as current* Collaborators: Not Provided	Study Start: September 14, 2018 Primary Completion: January 1, 2023 (Final data collection date for primary outcome measure) Study Completion: January 1, 2024 First Posted: July 27, 2018 Results First Posted: Last Update Posted: September 9, 2022

	NCT Number	Title	Other Names	Status	Conditions	Interventions	Characteristics	Population	Sponsor/Collaborators	Dates
19	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
20	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
21	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
22	NCT03190941	Administering Peripheral Blood Lymphocytes Transduced With a Murine T-Cell Receptor Recognizing the G12V Variant of Mutated RAS in HLA-A*11:01 Patients Study Documents:	Title Acronym: Other Ids: 170113 17-C-0113	Recruiting	• Pancreatic Cancer • Gastric Cancer • Gastrointestinal Cancer • Colon Cancer • Rectal Cancer	• Drug: Cyclophosphamide Days -7 and -6: Cyclophosphamide 60 mg/kg/day x 2 days IV in 250 mL D5W infused simultaneously with mesna 15 mg/kg/day over 1 hour x 2 days. • Drug: Fludarabine Days -7 to -3: Fludarabine 25 mg/m2/day IVPB daily over 30 minutes for 5 days. • Biological: Anti-KRAS G12V mTCR PBL Day 0: Cells will be infused intravenously on the Patient Care Unit over 20-30 minutes (2-4 days after the last dose of fludarabine). • Drug: Aldesleukin Aldesleukin 720,000 IU/kg IV (based on total body weight) over 15 minutes approximately every 8 hours beginning within 24 hours of cell infusion and continuing for up to 3 days (maximum 9 doses).	Study Type: Interventional Phase: Phase 1 Phase 2 Study Design: Allocation: Non-Randomized Intervention Model: Sequential Assignment Masking: None (Open Label) Primary Purpose: Treatment Primary Outcome Measures: • Response rate [Time Frame: 6 weeks (+/- 2 weeks) after cell infusion, then at week 12, every 3 months x3, every 6 months x2 years.] • Maximum Tolerated Dose [Time Frame: End of treatment] Secondary Outcome Measures: Survival and persistence of mTCR gene-engineered cells. [Time Frame: approximately 4-5 years]	Actual Enrollment: Estimated Enrollment: 110 Original Estimated Enrollment: *Same as current* Age: 18 Years to 70 Years (Adult, Older Adult) Sex: All	Study Sponsors: *Same as current* Collaborators: Not Provided	Study Start: September 21, 2017 Primary Completion: June 29, 2027 (Final data collection date for primary outcome measure) Study Completion: June 29, 2028 First Posted: June 19, 2017 Results First Posted: Last Update Posted: September 14, 2022

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