

Cell and Gene Therapy Catapult ATMP clinical trials database 2020

Sponsor	Title	Clinical Database Numbers	United Kingdom Site(s)	Trial Status	Trial Phase	Year Trial Started	Cell Type	Gene Modification/ Gene Therapy	Type of Virus Vector Used (if applicable)	Autologous/ Allogeneic	Disease Area	Indication
Keele University	Autologous Chondrocyte Transplantation/ Implantation Versus Existing Treatments	ISRCTN4891177	University Hospital of Wales University Hospital Keele University Hospital Birmingham University Hospital Southampton Wessex Institute of Technology	In follow-up	Phase III	2004	Chondrocytes	No	N/A	Autologous	Musculoskeletal	Unspecified Musculoskeletal Disorders
St. Jude Children's Research Hospital Inc	An Open Label Dose-escalation Study of a Self Complementary Adeno-associated Viral Vector (scAAV 2/8-LP1-hFIXco) for Gene Transfer in Hemophilia B	NCT00979238 2005-005711-17	Royal Free Hospital, London	Suspended	Phase I/II	2009	N/A	Yes, in vivo	AAV	N/A	Haematological	Hemophilia B (Factor IX Deficiency)
Axovant Gene Therapies Ltd	A Multicenter, Open-label Study to Determine the Long Term Safety, Tolerability and Efficacy of Prosvavin in Patients with Bilateral, Idiopathic Parkinson's Disease	NCT01856439 2009-017253-35	Addenbrooke's Hospital, Cambridge	In follow-up	Phase I/II	2010	N/A	Yes, in vivo	Lentivirus	N/A	Neurological	Parkinson's Disease
Boston Children's Hospital	Multi-institutional Phase I/II Trial Evaluating the Treatment of SCID-X1 Patients with Retrovirus-mediated Gene Transfer	NCT01129544	Great Ormond Street Hospital, London	In follow-up	Phase I/II	2010	CD34 and/or CD133 stem cells	Yes, ex vivo	Retrovirus	Autologous	Inflammatory and immune system	X-Linked SCID Severe Combined Immune Deficiency (SCID)
Great Ormond Street Hospital for Children NHS Trust / UCL Institute of Child Health	Gene Therapy For SCID-X1 Using A Self-inactivating (SIN) Gammaretroviral Vector	2007-000684-16	Great Ormond Street Hospital, London	Completed	Phase I/II	2010	CD34 and/or CD133 stem cells	Yes, ex vivo	Retrovirus	Autologous	Inflammatory and immune system	X-Linked SCID
ReNeuron Group Plc	A Phase I Safety Trial of CTX0E03 Drug Product Delivered Intracranially in the Treatment of Patients with Stable Ischemic Stroke	NCT01151124	Queen Elizabeth University Hospital, Glasgow	In follow-up	Phase I	2010	Neural	Yes, ex vivo	Retrovirus	Allogeneic	Cardiovascular	Ischemic Stroke
Great Ormond Street Hospital for Children NHS Trust	Gene Therapy for SCID-X1 Using a Self-inactivating (SIN) Gammaretroviral Vector	NCT01175239	Great Ormond Street Hospital, London	Terminated	Phase II	2011	CD34 and/or CD133 stem cells	Yes, ex vivo	Retrovirus	Autologous	Inflammatory and immune system	X-Linked Severe Combined Immune Deficiency (SCID)
University College London	CMV TCR Gene Therapy: A Phase I Safety, Toxicity and Feasibility Study of Adoptive Immunotherapy with CMV TCR-transduced Donor-derived T cells for Recipients of Allogeneic Haematopoietic Stem Cell Transplantation	NCT02988258 2008-006649-18	University Hospitals Birmingham NHS Foundation Trust University Hospitals Bristol NHS Foundation Trust University College London Hospitals Nottingham University Hospital	Terminated	Phase I	2011	T cells	Yes, ex vivo	Retrovirus	Allogeneic	Oncology (Haematology)	AML
Azellon Ltd	A Prospective Open-label Study to Evaluate the Safety of Cell Bandage (Mesenchymal Stem Cells) in the Treatment of Meniscal Tears	2010-024162-22	Southmead Hospital, Bristol	In follow-up	Phase I/II	2012	Mesenchymal stem/stromal cells	No	N/A	Autologous	Musculoskeletal	Knee Meniscus Repair
Great Ormond Street Hospital for Children NHS Trust	Phase I/II, Historical Controlled, Open-label, Non-randomised, Single-centre Trial to Assess the Safety and Efficacy of EF1αS-ADA Lentiviral Vector Mediated Gene Modification of Autologous CD34+ Cells From ADA-deficient Individuals	NCT01380990 2010-024253-36	Great Ormond Street Hospital, London	Completed	Phase I/II	2012	CD34 and/or CD133 stem cells	Yes, ex vivo	Lentivirus	Autologous	Inflammatory and immune system	Adenosine Deaminase (ADA) Deficiency Related SCID
Northwest Biotherapeutics Inc	A Phase III Clinical Trial Evaluating DCVax-L, Autologous Dendritic Cells Pulsed with Tumor Lysate Antigen for the Treatment of Glioblastoma Multiforme (GBM)	NCT00045968 2011-001977-13	Cambridge University Hospitals NHS Foundation Trust King's College Hospital NHS Foundation Trust, London University College London Hospitals Queen Elizabeth Hospital, Birmingham	Completed	Phase III	2012	Dendritic cells	No	N/A	Autologous	Oncology (Other)	Glioblastoma multiforme Brain Cancer
University of Cambridge	An Open Label Study to Assess the Safety and Efficacy of Neural Allo-Transplantation with Fetal Ventral Mesencephalic Tissue in Patients with Parkinson's Disease	NCT01898390	Cardiff University Imperial College London University College London Hospitals University of Cambridge	In follow-up	Phase I	2012	Neural	No	N/A	Allogeneic	Neurological	Parkinson's Disease
bluebird bio Inc	A Phase II/III Study of the Efficacy and Safety of Hematopoietic Stem Cells Transduced with Lenti-D Lentiviral Vector for the Treatment of Cerebral Adrenoleukodystrophy (CALD)	NCT01896102 2011-001953-10	Great Ormond Street Hospital for Children NHS Foundation Trust, London	In follow-up	Phase III/III	2013	CD34 and/or CD133 stem cells	Yes, ex vivo	Lentivirus	Autologous	Metabolic (Other)	Adrenoleukodystrophy (Adrenomyeloneuropathy/ Schilder-Addison Complex)
Genethon SA	A Phase I/II, Non Randomized, Multicenter, Open-label Study of Autologous CD34+ Cells Transduced with the G1XCGD Lentiviral Vector in Patients with X-linked Chronic Granulomatous Disease	NCT01855685 2012-000242-35	Great Ormond Street Hospital for Children NHS Foundation Trust, London Royal Free London NHS Foundation Trust University College London Hospitals	In follow-up	Phase I/II	2013	CD34 and/or CD133 stem cells	Yes, ex vivo	Lentivirus	Autologous	Inflammatory and immune system	Chronic Granulomatous Disease
Mesoblast Ltd	A Prospective, DB, Randomized, Placebo-controlled Clinical Trial of Intracoronary Infusion of Mesenchymal Precursor Cells (MPC) in the Treatment of Patients with ST-elevation Myocardial Infarction	NCT01781390 2010-020497-41	2 sites (not specified)	In follow-up	Phase II	2013	Mesenchymal stem/stromal cells	No	N/A	Allogeneic	Cardiovascular	Myocardial Infarction

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Queen Mary University of London	The Effect of Intracoronary Reinfusion of Bone Marrow-derived Mononuclear Cells (BM-MNC) on All Cause Mortality in Acute Myocardial Infarction	NCT01569178 2012-001495-11	King's College London Queen Mary University of London University College London Hospital New Cross Hospital, Wolverhampton	Completed	Phase III	2013	Bone marrow mononuclear cells	No	N/A	Autologous	Cardiovascular	Myocardial Infarction
Sotio AS	A Randomized, Double Blind, Multicenter, Parallel-group, Phase III Study to Evaluate Efficacy and Safety of DCVAC/PCa versus Placebo in Men with Metastatic Castration Resistant Prostate Cancer Eligible for first Line Chemotherapy	NCT02111577 2012-002814-38	The Clatterbridge Cancer Centre, Bebington University Hospitals Birmingham NHS Foundation Trust Bristol Haematology and Oncology Centre Cambridge University Hospitals NHS Foundation Trust University of Surrey, Guildford Royal Free Hospital, London The Royal Marsden NHS Foundation Trust, London The Christie NHS Foundation Trust, Manchester Freeman Hospital, Newcastle upon Tyne	Completed	Phase III	2013	Dendritic cells	No	N/A	Autologous	Oncology (Other)	Metastatic castrate-resistant prostate cancer
The Robert Jones and Agnes Hunt Orthopaedic and District Hospital NHS Trust	Autologous Cell Therapy for Osteoarthritis: an Evaluation of the Safety and Efficacy of Autologous Transplantation of Articular Chondrocytes and/or Bone Marrow-derived Stromal Cells to Repair Chondral/osteochondral Lesions of the Knee	ISRCTN98997175 2010-022072-31	The Robert Jones and Agnes Hunt Orthopaedic Hospital NHS Foundation Trust	Recruiting	Phase II	2013	Chondrocytes	No	N/A	Autologous	Musculoskeletal	Knee Osteoarthritis
Tissue Engineering Technologies AG	A Prospective Randomized Controlled Multicenter Phase-III Clinical Study to Evaluate the Safety and Effectiveness of Novocart 3D Plus Compared to the Standard Procedure Microfracture in the Treatment of Articular Cartilage Defects of the Knee	NCT01656902 2011-005798-22	Royal Devon and Exeter Hospital	In follow-up	Phase III	2013	Chondrocytes	No	N/A	Autologous	Musculoskeletal	Articular Cartilage Defect
University College London	Adoptive Immunotherapy with CD25/71 Allogeneic Donor T Cells to Improve Immunity After Unrelated Donor Stem Cell Transplant	NCT01827579 2013-000872-14	Manchester Royal Infirmary University College London Hospitals	Completed	Phase I/II	2013	T cells	No	N/A	Allogeneic	Oncology (Haematology)	AML
University of Birmingham	AdUP: A Phase I Clinical Trial of a Replication Defective Type 5 Adenovirus Vector Expressing Nitroreductase and GM-CSF (AdNRGM) Given Via Trans-perineal, Template-guided, Intra-prostatic Injection, Followed by Intravenous CB1954, in Patients with Locally Relapsed Hormone-refractory Prostate Cancer	NCT04374240	Queen Elizabeth Hospitals, Birmingham University of Warwick	In follow-up	Phase I	2013	N/A	Yes, in vivo	Adenovirus	N/A	Oncology (other)	Prostate Cancer
Amgen Inc	A Phase III, Multicenter, Trial of Talimogene Laherparepvec in Combination with Pembrolizumab (MK-3475) for Treatment of Unresectable Stage IIIB to IVM1c Melanoma (MASTERKEY-265/KEYNOTE-034)	NCT02263508	University Hospitals Birmingham NHS Foundation Trust Royal Surrey County Hospital St James's University Hospital Leeds Leicester Royal Infirmary Guy's and St. Thomas' NHS Foundation Trust The Royal Marsden Hospital London The Christie NHS Foundation Trust, Manchester Church Hospital Oxford Royal Preston Hospital Southampton General Hospital	In follow-up	Phase III	2014	N/A	Yes, in vivo	HSV-1	N/A	Oncology (Other)	Melanoma
Cook Group Inc	A Prospective Nonrandomized Study of Autologous Muscle Derived Cell (AMDC) Transplantation for Treatment of Fecal Incontinence	NCT01600755 2013-004672-35	Royal London Hospital	In follow-up	Phase I/II	2014	Other	No	N/A	Autologous	Gastrointestinal (Other)	Fecal Incontinence
Genethon SA	Long Term Safety Follow up of Patients Enrolled in the Phase I/II Clinical Trial of Haematopoietic Stem Cell Gene Therapy for the Wiskott-Aldrich Syndrome (GTG 002-07 and GTG 003-08)	NCT02333760 2014-000274-20	UCL Institute of Child Health, London	Recruiting	Phase I/II	2014	CD34 and/or CD133 stem cells	Yes, ex vivo	Lentivirus	Autologous	Inflammatory and immune system	Wiskott-Aldrich Syndrome
Adaptimmune Therapeutics Plc	A Phase I/II Dose Escalation Open Label Clinical Trial Evaluating the Safety and Efficacy of MAGE A10G796T in Subjects with Stage IIIB or Stage IV Non-Small Cell Lung Cancer (NSCLC)	NCT02592577	University College London Hospitals The Christie NHS Foundation Trust, Manchester Queen Elizabeth Hospital, Birmingham	In follow-up	Phase I	2015	T cells	Yes, ex vivo	Retrovirus	Autologous	Oncology (Haematology)	Non-Small Cell Lung Cancer Lung Cancer Non-Small Cell Lung Carcinoma Carcinomas
Bellicum Pharmaceuticals Inc	Phase II Extension Study of CaspaCIDE T Cells (BPX-501) From an HLA-partially Matched Family Donor after Negative Selection of TCR α and β ; +T Cells in Pediatric Patients Affected by Hematological Disorders	NCT02065869 2014-000584-41	Great Ormond Street Hospital, London Great North Children's Hospital, Newcastle Upon Tyne	Completed	Phase II	2015	T cells	Yes, ex vivo	Retrovirus	Allogeneic	Oncology (Haematology)	Haematological Malignancies
BioMarin Pharmaceutical Inc	A Phase I/II, Dose-escalation Safety, Tolerability and Efficacy Study of Valoctocogene Roxaparvoec, an Adenovirus-Associated Virus Vector-Mediated Gene Transfer of Human Factor VIII in Patients with Severe Haemophilia A	NCT02576795 2014-003880-38	Hampshire Hospitals NHS Foundation Trust, Basingstoke Queen Elizabeth Hospital, Birmingham University Hospitals Bristol NHS Foundation Trust Cambridge University Hospitals NHS Foundation Trust Barts Health NHS Trust, London Guy's & St. Thomas' NHS Foundation Trust, London Imperial College Healthcare NHS Trust, London University Hospital Southampton NHS Foundation Trust Greater Glasgow Health Board, Glasgow	In follow-up	Phase I/II	2015	N/A	Yes, in vivo	AAV	N/A	Haematological	Hemophilia A (Factor VIII Deficiency)

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bluebird bio inc	Long-term Follow-up of Subjects with Cerebral Adrenoleukodystrophy who were Treated with Lenti-D Drug Product	NCT02698579 2015-002805-13	Great Ormond Street Hospital, London	Recruiting	Phase III	2015	CD34 and/or CD133 stem cells	Yes, ex vivo	Lentivirus	Autologous	Metabolic (Other)	Adrenoleukodystrophy
CellProthera SAS	A Multicentric Controlled Phase I/IIb Study Evaluating the Safety and the Efficacy of in Vitro Expanded Peripheral Blood CD34+ Stem Cells Output by the StemXpand Automated Process, and Injected in Patients With an Acute Myocardial Infarction and a Left Ventricle Ejection Fraction (LVEF) Remaining Below or Equal to 45% After PTCA and Stent(s) Implantation Versus Standard of Care	NCT02669810	Leeds Teaching Hospitals NHS Trust Newcastle University University of Edinburgh Queen Elizabeth Hospital NHS Foundation Trust	Recruiting	Phase I/II	2015	CD34 and/or CD133 stem cells	No	N/A	Autologous	Cardiovascular	Myocardial Infarction Congestive Heart Failure (Heart Failure)
King's College London	Phase I/II Trial: T4 Immunotherapy of Head and Neck Cancer	NCT01818323	Guy's and St Thomas' NHS Foundation Trust, London	In follow-up	Phase I/II	2015	T cells	Yes, ex vivo	Retrovirus	Autologous	Oncology (Other)	Head And Neck Cancer
Kuur Therapeutics Ltd	A Phase II Single Arm Study to Investigate the Efficacy of Autologous EBV-specific T-cells for the Treatment of Patients with Aggressive EBV Positive Extranodal NK/T-cell Lymphoma (ENKTCL)	NCT01948180 2013-004380-31	University College London Hospitals The Christie Clinic, Manchester	Completed	Phase II	2015	T cells	No	N/A	Autologous	Oncology (Haematology)	EBV positive extranodal NK/T-cell lymphoma
Moorfields Eye Hospital NHS Foundation Trust	Phase I, Open-label, Safety and Feasibility Study of Implantation of PF-05206388 (Human Embryonic Stem Cell Derived Retinal Pigment Epithelium (RPE) Living Tissue Equivalent) in Subjects with Acute Wet Age Related Macular Degeneration and Recent Rapid Vision Decline	NCT01691261	Moorfields Eye Hospital, London	In follow-up	Phase I	2015	Retinal	No		Allogeneic	Ophthalmology	Acute Wet Age Related Macular Degeneration
TC BioPharm Ltd	Adaptive Study of the Safety, Tolerability and Efficacy of Autologous Gamma Delta T Lymphocyte Therapy (ImuniCell) in Patients with Advanced Cancers Refractory to Current Treatment or Have Indolent Disease for Which Immunotherapy May be Beneficial	NCT02459067 2015-000402-19	University Hospital of Wales Western General Hospital, Edinburgh Beatson West of Scotland Cancer Centre, Glasgow St. James's University Hospital, Leeds University College London Hospitals Churchill Hospital, Oxford Southampton General Hospital	Terminated	Phase II	2015	T cells	No	N/A	Autologous	Oncology (Other)	Non-Small Cell Lung Cancer
University of Oxford NIHR Evaluation Trials and Studies Coordinating Centre	An Open Label Phase II Clinical Trial of Retinal Gene Therapy for Choroideremia Using an Adeno-associated Viral Vector (AAV2) Encoding Rab-escort Protein 1 (REP1)	NCT02407678 2015-001383-18	Moorfields Eye Hospital NHS Foundation Trust, London Oxford University Hospitals NHS Foundation Trust	In follow-up	Phase II	2015	N/A	Yes, in vivo	AAV	N/A	Ophthalmology	Choroideremia
Amgen Inc	A Phase Ib/III Multicenter, Randomized, Trial of Talimogene Laherparepvec in Combination with Pembrolizumab for the Treatment of Subjects with Recurrent or Metastatic Squamous Cell Carcinoma of the Head and Neck	NCT02626000 2015-003011-38	University Hospitals Birmingham NHS Foundation Trust St Thomas' Hospital, London The Royal Marsden, London Churchill Hospital, Oxford Clatterbridge Hospital	Completed	Phase III	2016	N/A	Yes, in vivo	HSV-1	N/A	Oncology (Other)	Melanoma
Biogen Inc	A Randomized, Open Label, Outcomes-assessor Masked, Prospective, Parallel Controlled Group, Phase III Clinical Trial of Retinal Gene Therapy for Choroideremia Using an Adeno-associated Viral Vector (AAV2) Encoding Rab Escort Protein 1 (REP1)	NCT03496012 2015-003958-41	Manchester Royal Infirmary John Radcliffe Hospital Oxford	In follow-up	Phase III	2016	N/A	Yes, in vivo	AAV	N/A	Ophthalmology	Choroideremia
Cancer Research UK	A Cancer Research UK Phase I Trial of Anti-GD2 Chimeric Antigen Receptor (CAR) Transduced T-cells (1RG-CART) in Patients with Relapsed or Refractory Neuroblastoma	NCT02761915	Great Ormond Street Hospital for Children NHS Foundation Trust, London University College London Hospitals	Recruiting	Phase I	2016	T cells	Yes, ex vivo	Retrovirus	Autologous	Oncology (Other)	Relapsed or Refractory Neuroblastoma
GlaxoSmithKline Plc	A Pilot Study of Genetically Engineered NY-ESO-1 Specific NY-ESO-1(c259) T in HLA-A2+ Patients with Synovial Sarcoma	NCT01343043 2015-005594-21	The Christie NHS Foundation Trust, Manchester University College London Hospitals	Completed	Phase I/II	2016	T cells	Yes, ex vivo	Lentivirus	Autologous	Oncology (Other)	Synovial Sarcoma
Institut de Recherches Internationales Servier	Long-term Follow-up Study of Patients Who Have Previously Been Exposed to UCART19 (Allogeneic Engineered T-cells Expressing a Lentiviral-based Anti-CD19 Chimeric Antigen Receptor)	NCT02735083 2016-000297-38	The Christie NHS Foundation Trust, Manchester King's College Hospital NHS Foundation Trust, London Great Ormond Street Hospital, London	Recruiting	Phase I	2016	T cells	Yes, ex vivo	Lentivirus	Allogeneic	Oncology (Haematology)	Advanced Lymphoid Malignancies
King's College London Guy's and St Thomas' NHS Foundation Trust	A Double-blind, Placebo Controlled, First into Human Clinical Trial of T Regulatory Cells (TR004) for Inflammatory Bowel Disease Using (Ex Vivo) Treg Expansion	NCT03185000	Guy's Hospital, London St Thomas' Hospital, London	Recruiting	Phase I/II	2016	T cells	No	N/A	Autologous	Gastrointestinal (Other)	Crohn's Disease

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MeiraGTx Holdings Plc	Long-Term Follow-Up Study of Participants Following an Open Label, Multi-Centre, Phase I/II Dose Escalation Trial of an Adeno-Associated Virus Vector (AAV2/5-OPTRPE65) for Gene Therapy of Adults and Children with Retinal Dystrophy Owing to Defects in RPE65 (LCA2)	NCT02946879 2016-000898-20	Moorfields Eye Hospital	Recruiting	Phase I/II	2016	N/A	Yes, in vivo	AAV	N/A	Ophthalmology	Leber Congenital Amaurosis (LCA) Unspecified Ophthalmological Disorders
MeiraGTx Holdings Plc	An Open Label, Multi-centre, Phase I/II Dose Escalation Trial of a Recombinant Adeno-associated Virus Vector (AAV2/8-hCARp-hCNGB3) for Gene Therapy of Adults and Children with Achromatopsia Owing to Defects in CNGB3	NCT03001310 2016-002290-35	Moorfields Eye Hospital, London	Completed	Phase I/II	2016	N/A	Yes, in vivo	AAV	N/A	Ophthalmology	Achromatopsia
Mina Therapeutics Ltd	A First-in-human, Multi-center, Open-label, Phase Ib/IIa Clinical Study with RNA Oligonucleotide Drug MTL-CEBPA to Investigate Its Safety and Tolerability in Patients with Advanced Liver Cancer	NCT02716012	University Hospitals Birmingham NHS Foundation Trust Cambridge University Hospitals NHS Trust The Beatson West of Scotland Cancer Centre Clatterbridge Cancer Centre NHS Foundation Trust Guy's and St. Thomas' NHS Foundation Trust Imperial College Healthcare NHS Trust University College London Hospitals Newcastle upon Tyne Hospitals NHS Foundation Trust	In follow-up	Phase I	2016	N/A	Yes, in vivo	Non-viral	N/A	Oncology (Other)	Hepatocellular Carcinoma
Servier Institute of International Research	Phase Ib, Open Label, Dose-escalation Study Followed by a Safety Expansion Part to Evaluate the Safety, Expansion and Persistence of a Single Dose of UCART19 (Allogeneic Engineered T-cells Expressing Anti-CD19 Chimeric Antigen Receptor), Administered Intravenously in Patients with Relapsed or Refractory CD19 Positive B-cell Acute Lymphoblastic Leukaemia (B-ALL)	NCT02746952 2016-000296-24	The Christie NHS Foundation Trust, Manchester King's College Hospital NHS Foundation Trust	Completed	Phase I	2016	T cells	Yes, ex vivo	Lentivirus	Allogeneic	Oncology (Haematology)	B-cell acute lymphoblastic leukaemia (B-ALL)
Servier Institute of International Research	A Phase I, Open Label, Non-comparative Study to Evaluate the Safety and the Ability of UCART19 to Induce Molecular Remission in Paediatric Patients with Relapsed/Refractory B-cell Acute Lymphoblastic Leukaemia	NCT02808442 2015-004293-15	Great Ormond Street Hospital, London	Withdrawn	Phase I	2016	T cells	Yes, ex vivo	Lentivirus	Allogeneic	Oncology (Haematology)	Relapsed/Refractory B Acute Lymphoblastic Leukemia
The University of Edinburgh	Macrophage Therapy for Liver Cirrhosis (MATCH)	2015-000963-15	Edinburgh Royal Infirmary Glasgow Royal Infirmary Ninewells Hospital, Dundee	In follow-up	Phase I/II	2016	Other	No	N/A	Autologous	Gastrointestinal (Liver)	Advanced Liver Cirrhosis
University College London	Immunotherapy with CD19 CAR Redirected T-cells for High Risk, Relapsed Pediatric CD19+ Acute Lymphoblastic Leukaemia and Other Hematological Malignancies	NCT02443831	Royal Manchester Children's Hospital Great Ormond Street Hospital for Children NHS Foundation Trust, London University College London Hospitals Manchester Royal Infirmary	In follow-up	Phase I	2016	T cells	Yes, ex vivo	Lentivirus	Autologous	Oncology (Haematology)	ALL
University College London	COBAL: Evaluation of CAR19 T-cells as an Optimal Bridge to Allogeneic Transplantation	NCT02431988	University College London Hospitals	In follow-up	Phase I	2016	T cells	Yes, ex vivo	Lentivirus	Autologous	Oncology (Other)	Diffuse Large B-Cell Lymphoma
Adaptimmune Therapeutics Plc	A Phase I Open Label Clinical Trial Evaluating the Safety and Anti-tumor Activity of Autologous T Cells Expressing Enhanced TCRs Specific for Alpha Fetoprotein (AFP332T) in HLA-A2 Positive Subjects with Advanced Hepatocellular Carcinoma (HCC) or Other AFP Expressing Tumor Types	NCT03132792	Beatson West of Scotland Cancer Centre Guy's and St Thomas' NHS Foundation Trust University College London Hospitals - Clinical Research Facility The Christie NHS Foundation Trust, Manchester	Recruiting	Phase I	2017	T cells	Yes, ex vivo	Lentivirus	Autologous	Oncology (Other)	Hepatocellular Carcinoma Liver Cancer
Audentes Therapeutics Inc	A Phase I/II, Randomized, Open-label, Ascending-dose, Delayed-treatment Concurrent Control Clinical Study to Evaluate the Safety and Preliminary Efficacy of AT342, an AAV8-delivered Gene Transfer Therapy in Crigler-Najjar Syndrome Subjects Aged 1 Year and Older (VALENS)	NCT03223194 2017-001631-39	Sites not specified	Suspended	Phase I/II	2017	N/A	Yes, in vivo	AAV	N/A	Metabolic (Other)	Crigler-Najjar Syndrome
Audentes Therapeutics Inc	ASPIRO: A Phase 1/2, Randomized, Open-Label, Ascending-Dose, Delayed-Treatment Concurrent Control Clinical Study to Evaluate the Safety and Efficacy of AT132, an AAV8-Delivered Gene Therapy in X-Linked Myotubular Myopathy (XLMTM) Patients	NCT03199469 2017-000876-27	Great Ormond Street Hospital, London	Recruiting	Phase I/II	2017	N/A	Yes, in vivo	AAV	N/A	Neuromuscular	X-Linked Myotubular Myopathy (XLMTM or MTM)
Autolus Therapeutics Plc	A Single Arm, Open-label, Multi-centre, Phase I/II Study Evaluating the Safety and Clinical Activity of AUTO3, a CAR T Cell Treatment Targeting CD19 and CD22 with Anti PD1 Antibody in Patients with Relapsed or Refractory Diffuse Large B Cell Lymphoma	NCT03287817 2016-004682-11	Freeman Hospital, Newcastle upon Tyne The Christie NHS Foundation Trust, Manchester University College London Hospitals NHS Foundation Trust The Beatson West of Scotland Cancer Centre / Queen Elizabeth University Hospital, Glasgow	Recruiting	Phase I/II	2017	T cells	Yes, ex vivo	Retrovirus	Autologous	Oncology (Haematology)	B-Cell lymphoma

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Autolus Therapeutics Plc	A Single-arm, Open-label, Multi-centre, Phase I/II Study Evaluating the Safety and Clinical Activity of AUTO3, a CAR T Cell Treatment Targeting CD19 and CD22 in Paediatric and Young Adult Patients with Relapsed or Refractory B Cell Acute Lymphoblastic Leukaemia	NCT03289455 2016-004680-39	Great Ormond Street Hospital for Children NHS Foundation Trust, London University College London Hospitals NHS Foundation Trust Royal Manchester Children's Hospital	Completed	Phase I/II	2017	T cells	Yes, ex vivo	Retrovirus	Autologous	Oncology (Haematology)	ALL
Autolus Therapeutics Plc	A Single-arm, Open-label, Multi-center, Phase I/II Study Evaluating the Safety and Clinical Activity of AUTO2, a CAR T Cell Treatment Targeting BCMA and TACI, in Patients with Relapsed or Refractory Multiple Myeloma	NCT03287804 2016-003893-42	University College London Hospitals NHS Foundation Trust The Christie NHS Foundation Trust, Manchester Freeman Hospital, Newcastle upon Tyne	Terminated	Phase I/II	2017	T cells	Yes, ex vivo	Retrovirus	Autologous	Oncology (Haematology)	Multiple Myeloma (Kahler Disease)
Bellicum Pharmaceuticals Inc	Follow-up of Phase I/II Study of CaspaCIDE T Cells (BPX-501) From an HLA-partially Matched Family Donor After Negative Selection of TCR α & β ; +T Cells in Pediatric Patients Affected by Hematological Disorders	NCT03733249 2016-003226-16	Great Ormond Street Hospital, London Great North Children's Hospital, Newcastle Upon Tyne	Recruiting	Phase I/II	2017	T cells	Yes, ex vivo	Retrovirus	Autologous	Oncology (Haematology)	AML
Biogen Inc	A Dose Escalation (Phase I), and Dose Expansion (Phase II/III) Clinical Trial of Retinal Gene Therapy for X-linked Retinitis Pigmentosa Using an Adeno-Associated Viral Vector (AAV8) Encoding Retinitis Pigmentosa GTPase Regulator (RPGR)	NCT03116113 2016-003852-60	Manchester Royal Eye Hospital Oxford Eye Hospital Southampton site (not specified)	Recruiting	Phase II/III	2017	N/A	Yes, in vivo	AAV	N/A	Ophthalmology	Retinitis Pigmentosa (Retinitis)
BioMarin Pharmaceutical Inc	A Phase III Open-Label, Single-Arm Study To Evaluate The Efficacy and Safety of BMN 270, an Adeno-Associated Virus Vector-Mediated Gene Transfer of Human Factor VIII in Hemophilia A Patients With Residual FVIII Levels \leq 1 IU/dL Receiving Prophylactic FVIII Infusions	NCT03370913 2017-003215-19	Queen Elizabeth Hospital, Birmingham Addenbrooke's Hospital, Cambridge Glasgow Royal Infirmary, Glasgow Barts and The London School of Medicine and Dentistry St Thomas' Hospital, London Churchill Hospital, Oxford University Hospital Southampton NHS Foundation Trust Royal Cornwall Hospital, Truro	In follow-up	Phase III	2017	N/A	Yes, in vivo	AAV	N/A	Haematological	Haemophilia A
bluebird bio Inc	A Phase III Single Arm Study Evaluating the Efficacy and Safety of Gene Therapy in Subjects with Transfusion-dependent β -Thalassemia, Who Do Not Have a β & β ; 0/ β & β ; 0 Genotype, by Transplantation of Autologous CD34+ Stem Cells Transduced Ex Vivo with a Lentiviral β -A-T87Q-Globin Vector in Subjects \leq 50 Years of Age	NCT02906202 2015-004122-33	University College London Hospitals	In follow-up	Phase III	2017	CD34 and/or CD133 stem cells	Yes, ex vivo	Lentivirus	Autologous	Haematological	β -Thalassemia
bluebird bio Inc	A Phase III Single Arm Study Evaluating the Efficacy and Safety of Gene Therapy in Subjects with Transfusion-dependent β -Thalassemia by Transplantation of Autologous CD34+ Stem Cells Transduced Ex Vivo with a Lentiviral β -A-T87Q-globin Vector in Subjects \leq 50 Years of Age	NCT03207009 2016-003611-35	University College London Hospitals	Recruiting	Phase III	2017	CD34 and/or CD133 stem cells	Yes, ex vivo	Lentivirus	Autologous	Haematological	β -Thalassemia
Cancer Vaccines Ltd	A Phase I Clinical Study to Determine the Optimal Dose for the Safe Immune Restoration and Immune Response of Allogeneic Cell Immunotherapy (ACIT-1) in Adult Cancer Patients	NCT03096093	Royal Liverpool University Hospital The Clatterbridge Cancer Centre NHS Foundation Trust, Bebington	Recruiting	Phase I/II	2017	Antigen presenting cells	No		Allogeneic	Oncology (Other)	Pancreatic cancer
Cynata Therapeutics Ltd	An Open-label Phase I Study to Investigate the Safety and Efficacy of CYP-001 for the Treatment of Adults with Steroid-resistant Acute Graft Versus Host Disease	NCT02923375	University Hospitals Bristol NHS Foundation Trust Leeds Teaching Hospitals NHS Trust Liverpool University Hospitals NHS Foundation Trust Manchester University NHS Foundation Trust Nottinghamshire Healthcare NHS Foundation Trust	In follow-up	Phase I	2017	Mesenchymal stem/stromal cells	No	N/A	Allogeneic	Inflammatory and immune system	Graft Versus Host Disease (GVHD)
Eyeveensys SAS	A Phase I/II, Open-label, Multicenter, Dose Escalation Study Assessing Safety/ Tolerability of pEYS606 When Administered by Electrotransfer (ET) in Ciliary Muscle of Patients with Non-infectious Posterior, Intermediate or Panuveitis	NCT03308045	Bristol Eye Hospital Moorfields Eye Hospital, London	Recruiting	Phase I/II	2017	N/A	Yes, in vivo	Plasmid	N/A	Ophthalmology	Posterior Uveitis Intermediate Uveitis
GenSight Biologics SA	A Phase I/IIa, Open-label, Non-randomized, Dose-escalation Study to Evaluate the Safety and Tolerability of GS030 in Subjects with Retinitis Pigmentosa	NCT03326336 2017-002204-27	Moorfields Eye Hospital NHS Foundation Trust	Recruiting	Phase I/II	2017	N/A	Yes, in vivo	AAV	N/A	Ophthalmology	Retinitis Pigmentosa (Retinitis)

Sponsor	Title	Clinical Database Numbers	United Kingdom Site(s)	Trial Status	Trial Phase	Year Trial Started	Cell Type	Gene Modification/ Gene Therapy	Type of Virus Vector Used (if applicable)	Autologous/ Allogeneic	Disease Area	Indication
Great Ormond Street Hospital for Children NHS Trust	Efficacy and Safety of a Cryopreserved Formulation of Autologous CD34+ Haematopoietic Stem Cells Transduced ex Vivo With Elongation Factor 1 α ; Short Form (EFS) Lentiviral Vector Encoding for Human ADA Gene in Subjects With Severe Combined Immunodeficiency (SCID) Due to Adenosine Deaminase Deficiency	NCT03765632 2017-001275-23	Great Ormond Street Hospital for Children NHS Foundation Trust, London	Recruiting	Phase I/II	2017	CD34 and/or CD133 stem cells	Yes, ex vivo	Lentivirus	Autologous	Inflammatory and immune system	Severe Combined Immunodeficiency Due to ADA Deficiency
Iovance Biotherapeutics Inc	A Phase II, Multicenter Study to Assess the Efficacy and Safety of Autologous Tumor Infiltrating Lymphocytes (LN-144) for Treatment of Patients with Metastatic Melanoma	NCT02360579 2017-000760-15	Royal Marsden NHS Trust, London Addenbrooke's Hospital, Cambridge Sarah Cannon Research Institute, London Beatson West of Scotland Cancer Centre, Glasgow	In follow-up	Phase II	2017	T cells	No	N/A	Autologous	Oncology (Other)	Metastatic Melanoma
Iovance Biotherapeutics Inc	A Phase II, Multicenter Study to Evaluate the Efficacy and Safety Using Autologous Tumor Infiltrating Lymphocytes (LN-145) in Patients with Recurrent, Metastatic or Persistent Cervical Carcinoma	NCT03108495 2016-003447-11	Bristol Haematology and Oncology Centre Sarah Cannon Research Institute, London University College London Hospitals NHS Foundation Trust NHS Greater Glasgow and Clyde	Recruiting	Phase II	2017	T cells	No	N/A	Autologous	Oncology (Other)	Cervical Carcinomas
Kiadis Pharma NV	A Phase III, Multicenter, Randomized Controlled Study to Compare Safety and Efficacy of a Haploidentical HSCT and Adjunctive Treatment with ATIR101, a T-lymphocyte Enriched Leukocyte Preparation Depleted Ex Vivo of Host Alloreactive T-cells, Versus a Haploidentical HSCT with Post-transplant Cyclophosphamide in Patients with a Hematologic Malignancy	NCT02999854 2016-004672-21	St James University Hospital, Leeds Royal Liverpool University Hospital Hammersmith Hospital, London Manchester Royal Infirmary	In follow-up	Phase III	2017	T cells	No	N/A	Allogeneic	Oncology (Haematology)	ALL
Mario Negri Institute for Pharmacological Research	Novel Stromal Cell Therapy for Diabetic Kidney Disease (NEPHSTROM Study)	NCT02585622 2016-000661-23	Belfast Health and Social Care Trust, Belfast City Hospital University Hospitals Birmingham NHS Foundation Trust	Recruiting	Phase I/II	2017	Mesenchymal stem/stromal cells	No	N/A	Allogeneic	Renal and Urogenital	Type 2 Diabetes Diabetic Nephropathy
MeiraGTx Holdings Plc	An Open Label, Multi-centre, Phase I/II Dose Escalation Trial of a Recombinant Adeno-associated Virus Vector (AAV2-.RPGR) for Gene Therapy of Adults and Children with X-linked Retinitis Pigmentosa Owing to Defects in Retinitis Pigmentosa GTPase Regulator (RPGR)	NCT03252847 2016-003967-21	Moorfields Eye Hospital NHS Foundation Trust London Leeds Teaching Hospitals NHS Trust	In follow-up	Phase I/II	2017	N/A	Yes, in vivo	AAV	N/A	Ophthalmology	Retinitis Pigmentosa (Retinitis)
MeiraGTx Holdings Plc	Long-term Follow-up Study of Participants Following an Open Label, Multi-centre, Phase I/II Dose Escalation Trial of a Recombinant Adeno-associated Virus Vector (AAV2/8-hCARp.hCNGB3 and AAV2/8-hG1.7p.coCNGA3) for Gene Therapy of Adults and Children with Achromatopsia Owing to Defects in CNGB3 or CNGA3	NCT03278873 2016-003856-59	Moorfields Eye Hospital NHS Foundation Trust, London	Recruiting	Phase I/II	2017	N/A	Yes, in vivo	AAV	N/A	Ophthalmology	Achromatopsia
Montpellier University Hospital	A Phase IIb, Prospective, Multicentre, Double-blind, Triple-arm, Randomized Versus Placebo Trial, to Assess the Efficacy of a Single Injection of Either 2 or 10 X 10 ⁶ Autologous Adipose Derived Mesenchymal Stromal Cells (ASC) in the Treatment of Mild to Moderate Osteoarthritis (OA) of the Knee, Active and Unresponsive to Conservative Therapy for at Least 12 Months	NCT02838069 2015-002125-19	Cambridge University Hospitals NHS Foundation Trust	Recruiting	Phase II	2017	Adipose-derived stem cells	No	N/A	Autologous	Musculoskeletal	Osteoarthritis (knee)
Pluristem Therapeutics Inc	Efficacy, Tolerability and Safety of Intramuscular Injections of PLX PAD for the Treatment of Subjects with Critical Limb Ischemia (CLI) with Minor Tissue Loss Who Are Unsuited for Revascularization	NCT03006770 2015-005532-18	Hull And East Yorkshire Hospitals NHS Trust, Hull St George's Hospital, London Kings College Hospital, London Southmead Hospital, Bristol	Recruiting	Phase III	2017	Mesenchymal stem/stromal cells	No	N/A	Allogeneic	Cardiovascular	Critical Limb Ischemia (CLI)
Replimune Ltd	An Open-label, Multicenter, Phase I/II Study of RP1 as a Single Agent and in Combination with PD1 Blockade in Patients with Solid Tumors	NCT03767348 2016-004548-12	University of Leeds Teaching Hospital St George's Hospital, London Beatson West of Scotland Cancer Centre, Glasgow The Clatterbridge Cancer Centre NHS Foundation Trust, Bebington Royal Marsden Hospital, London	In follow-up	Phase I/II	2017	N/A	Yes, in vivo	HSV-1	N/A	Oncology (Other)	Advanced solid tumours
Rexgenero Ltd	The Efficacy and Safety of Intra-arterial Administration of REX-001 to Treat Ischemic Rest Pain in Subjects with Critical Limb Ischemia (CLI) Rutherford Category 4 and Diabetes Mellitus (DM): A Pivotal, Placebo-controlled, Double-blind, Parallel-group, Adaptive Trial	NCT03111238 2016-000240-34	Addenbrooke's Hospital, Cambridge University Hospital of Wales, Cardiff	Recruiting	Phase III	2017	Bone marrow mononuclear cells	No	N/A	Autologous	Cardiovascular	Critical Limb Ischemia in patients with Diabetes Mellitus

Sponsor	Title	Clinical Database Numbers	United Kingdom Site(s)	Trial Status	Trial Phase	Year Trial Started	Cell Type	Gene Modification/ Gene Therapy	Type of Virus Vector Used (if applicable)	Autologous/ Allogeneic	Disease Area	Indication
Rexgenero Ltd	The Efficacy and Safety of Intra-arterial Administration of REX-001 to Treat Ischemic Ulcers in Subjects with Critical Limb Ischemia (CLI) Rutherford Category 5 and Diabetes Mellitus: A Pivotal, Placebo-controlled, Double-blind, Parallel-group, Adaptive Trial	NCT03174522 2016-003980-21	Addenbrooke's Hospital, Cambridge University Hospitals Birmingham NHS Foundation Trust University Hospital of Wales Cardiff	Recruiting	Phase III	2017	Bone marrow mononuclear cells	No	N/A	Autologous	Cardiovascular	Critical Limb Ischemia in patients with Diabetes Mellitus
Ultragenyx Pharmaceutical Inc	A Phase I/II, Open-label Safety and Dose-finding Study of Adeno-associated Virus (AAV) Serotype 8 (AAV8)-mediated Gene Transfer of Human Ornithine Transcarbamylase (OTC) in Adults with Late-onset OTC Deficiency	NCT02991144 2016-001057-40	Queen Elizabeth Hospital, Birmingham National Hospital for Neurology and Neurosurgery	Recruiting	Phase I/II	2017	N/A	Yes, in vivo	AAV	N/A	Metabolic (Other)	Ornithine-Transcarbamylase Deficiency
Ultragenyx Pharmaceutical Inc	A Long-term Follow-up Study to Evaluate the Safety, Tolerability, and Efficacy of Adeno-associated Virus (AAV) rh10-mediated Gene Transfer of Human Factor IX in Adults with Moderate/severe to Severe Hemophilia B	NCT02971969 2016-003430-25	Haemostasis & Thrombosis Centre, Basingstoke Manchester Haemophilia Comprehensive Care Centre	In follow-up	Phase I/II	2017	N/A	Yes, in vivo	AAV	N/A	Haematological	Hemophilia B (Factor IX Deficiency)
University College London	GO-8: Gene Therapy for Haemophilia a Using a Novel Serotype 8 Capsid Pseudotyped Adeno-associated Viral Vector Encoding Factor VIII-V3	NCT03001830 2016-000925-38	Royal Free London NHS Foundation Trust	Recruiting	Phase I/II	2017	Other	Yes, in vivo	AAV	N/A	Haematological	Hemophilia A (Factor VIII Deficiency)
University College London	A Phase I/II, Open Label, Multicentre, Ascending Single Dose, Safety Study of a Novel Adeno-Associated Viral Vector (FLT180a) in Patients with Haemophilia B	NCT03369444 2017-000852-24	Basingstoke Haemostasis and Thrombosis Centre Guy's and St Thomas's NHS Foundation Trust, London Newcastle Hospitals NHS Foundation Trust Royal Free Hospital, London Oxford University Hospital University Hospital Southampton East Kent Hospitals University, Canterbury University of Sheffield Manchester Royal Infirmary	Recruiting	Phase I/II	2017	N/A	Yes, in vivo	AAV	N/A	Haematological	Hemophilia B (Factor IX Deficiency)
University College London	Immunotherapy for High Risk/Relapsed CD19+ Acute Lymphoblastic Leukaemia, B-cell Non-Hodgkin's Lymphoma (B-NHL) and Chronic Lymphocytic Leukaemia (CLL)/ Small Lymphocytic Lymphoma (SLL) Using CAR T-cells to Target CD19	NCT02935257	University College London Hospitals The Christie NHS Foundation Trust, Manchester The Beatson West of Scotland Cancer Centre / Queen Elizabeth University Hospital, Glasgow	In follow-up	Phase I	2017	T cells	Yes, ex vivo	Lentivirus	Autologous	Oncology (Haematology)	Acute Lymphocytic Leukemia (ALL, Acute Lymphoblastic Leukemia) Lymphoma B-Cell Non-Hodgkin Lymphoma Chronic Lymphocytic Leukemia (CLL)
University College London	Chimeric Antigen Receptor (CAR)19 Donor Lymphocytes for Relapsed Cluster of Differentiation (CD)19+ Malignancies Following Allogeneic Transplantation (CARD)	NCT02893189	University College London Hospitals	In follow-up	Phase I	2017	T cells	Yes, ex vivo	Unknown	Autologous	Oncology (Other)	Unspecified cancers
University of Leeds	A Prospective, Randomized, Single-centre Feasibility Study of Combined Autologous Platelet-rich Plasma and Concentrated Autologous Bone Marrow in Adult Patients with a Fresh Unilateral Tibial Diaphyseal Fracture Treated with Either Fine Wire Ring Fixator Device (Ilizarov) or Reamed Intramedullary Nailing	NCT03100695	Leeds Teaching Hospitals NHS Foundation Trust	Recruiting	Phase II	2017	N/A	No	N/A	Autologous	Musculoskeletal	Bone Fracture
Amphera BV	A Randomized, Open-label Phase II/III Study with Dendritic Cells Loaded with Allogeneic Tumour Cell Lysate (Pheralys) in Subjects with Mesothelioma as Maintenance Treatment (Mesopher) after Chemotherapy	NCT03610360 2017-001774-41	University of Leicester Queen Elizabeth Hospital, Birmingham	Recruiting	Phase II/III	2018	Antigen presenting cells	No	N/A	Autologous	Oncology (Other)	Malignant Pleural Mesothelioma Malignant Mesothelioma
Astellas Pharma Inc	A Safety Surveillance Study of Events of Special Interest Occurring in Subjects with Macular Degenerative Disease Treated with Human Embryonic Stem Cell-derived Retinal Pigment Epithelial Cell Therapy	NCT03167203	London site (not specified)	Recruiting	Phase I/II	2018	Retinal	No	N/A	Allogeneic	Ophthalmology	Age Related Macular Degeneration Dry (Atrophic) Macular Degeneration
Autolus Therapeutics Plc	Long-term Follow-up of Patients Previously Treated with Autologous T Cells Genetically Modified with Viral Vectors	NCT03628612 2016-004867-38	Great Ormond Street Hospital for Children NHS Foundation Trust, London University College London Hospitals Royal Manchester Children's Hospital Freeman Hospital, Newcastle upon Tyne	Recruiting	Phase II	2018	T cells	Yes, ex vivo	Retrovirus	Autologous	Oncology (Other)	Multiple Myeloma
Autolus Therapeutics Plc	A Single Arm, Open Label, Multi-centre, Phase I/II Study Evaluating the Safety and Clinical Activity of AUTO4, a CAR T-cell Treatment Targeting TRBC1, in Patients with Relapsed or Refractory TRBC1 Positive Selected T Cell Non-hodgkin Lymphoma	NCT03590574 2017-001965-26	The Christie NHS Foundation Trust, Manchester Freeman Hospital, Newcastle upon Tyne Manchester Royal Infirmary University College London Hospitals The Beatson West of Scotland Cancer Centre / Queen Elizabeth University Hospital, Glasgow	Recruiting	Phase I/II	2018	T cells	Yes, ex vivo	Retrovirus	Autologous	Oncology (Haematology)	T-Cell Lymphomas Peripheral T-Cell Lymphomas (PTCL) Non-Hodgkin Lymphoma Angioimmunoblastic T-Cell Lymphoma (AITL)/Immunoblastic Lymphadenopathy Anaplastic Large Cell Lymphoma (ALCL)

Sponsor	Title	Clinical Database Numbers	United Kingdom Site(s)	Trial Status	Trial Phase	Year Trial Started	Cell Type	Gene Modification/ Gene Therapy	Type of Virus Vector Used (if applicable)	Autologous/ Allogeneic	Disease Area	Indication
AveXis, Inc.	European, Phase III, Open-label, Single-arm, Single-dose Gene Replacement Therapy Clinical Trial for Patients with Spinal Muscular Atrophy Type 1 with One or Two SMN2 Copies Delivering AVXS-101 by Intravenous Infusion	NCT03461289 2017-000266-29	Great Ormond Street Hospital, London The John Walton Muscular Dystrophy Research Centre, Newcastle The Great North Children's Hospital, Newcastle upon Tyne	In follow-up	Phase III	2018	N/A	Yes, in vivo	AAV	N/A	Neuromuscular	Spinal Muscular Atrophy
AveXis, Inc.	A Global Study of a Single, One-time Dose of AVXS-101 Delivered to Infants with Genetically Diagnosed and Pre-symptomatic Spinal Muscular Atrophy with Multiple Copies of SMN2	NCT03505099 2017-004087-35	Great Ormond Street Hospital, London	In follow-up	Phase III	2018	N/A	Yes, in vivo	AAV	N/A	Neuromuscular	Spinal Muscular Atrophy
Axovant Gene Therapies Ltd	A Phase I/II Safety and Dose Evaluation Study of OXB-102 in Patients with Bilateral Idiopathic Parkinson's Disease	NCT03720418	University of Cambridge, Centre for Brain Repair The National Hospital for Neurology and Neurosurgery, London University College London Hospitals National Institute for Health Research, Southampton	Recruiting	Phase II	2018	N/A	Yes, in vivo	Lentivirus	N/A	Neurological	Parkinson's Disease
Barts Health NHS Trust	Phase II Study Assessing the Combined Use of Autologous Bone Marrow Derived Mononuclear Cells and G-CSF With Percutaneous Circulatory Assistance in the Treatment of Dilated Cardiomyopathy	NCT03572660 2018-001063-23	St Bartholomew's Hospital, London	Recruiting	Phase II	2018	Bone marrow mononuclear cells	No	N/A	Autologous	Cardiovascular	Dilated Cardiomyopathy
Bayer AG	A Phase I/II Open-label Safety and Dose-finding Study of BAY2599023 (DTX201), an Adeno-associated Virus (AAV) hu37-mediated Gene Transfer of B-domain Deleted Human Factor VIII, in Adults with Severe Hemophilia A	NCT03588299 2017-000806-39	St Thomas' Hospital London Manchester Royal Infirmary	Recruiting	Phase I/II	2018	N/A	Yes, in vivo	AAV	N/A	Haematological	Hemophilia A (Factor VIII Deficiency)
Biogen Inc	A Long-term Follow-up Study to Evaluate the Safety and Efficacy of Retinal Gene Therapy in Subjects with Choroideremia Treated Previously with Adeno-associated Viral Vector Encoding Rab Escort Protein-1 (AAV2-REP1) in an Antecedent Study	NCT03584165 2017-003104-42	2 sites (not specified)	Recruiting	Phase III	2018	N/A	Yes, in vivo	AAV	N/A	Ophthalmology	Choroideremia
BioMarin Pharmaceutical Inc	Phase III Study To Evaluate Efficacy/Safety of Valoctocogene Roxaparovec an AAV Vector-Mediated Gene Transfer of hFVIII at a Dose of 4E13vg/kg in Hemophilia A Patients with Residual FVIII Levels <=1IU/dL Receiving Prophylactic FVIII Infusions	NCT03392974 2017-003573-34	Addenbrookes Hospital, Cambridge Glasgow Royal Infirmary Barts and The London School of Medicine and Dentistry Hammersmith Hospital, London St Thomas' Hospital, London Churchill Hospital, Oxford Hemophilia and Thrombosis Centre University Hospital Southampton NHS Foundation Trust Queen Elizabeth Hospital NHS Foundation Trust	In follow-up	Phase III	2018	N/A	Yes, in vivo	AAV	N/A	Haematological	Hemophilia A (Factor VIII Deficiency)
BioMarin Pharmaceutical Inc	A Phase I/II Safety, Tolerability, and Efficacy Study of Valoctocogene Roxaparovec, an Adeno-associated Virus Vector-mediated Gene Transfer of Human Factor VIII in Hemophilia A Patients with Residual FVIII Levels <= 1 IU/dL and Pre-existing Antibodies Against AAV5	NCT03520712 2017-000662-29	Royal Free Hospital, London University Hospital Southampton NHS Foundation Trust	Recruiting	Phase I/II	2018	N/A	Yes, in vivo	AAV	N/A	Haematological	Hemophilia A (Factor VIII Deficiency)
Boston Children's Hospital	Phase I/II Trial of Lentiviral Gene Transfer for SCID-X1 with Low Dose Targeted Busulfan Conditioning	NCT03311503	Great Ormond Street Hospital, London	Recruiting	Phase I/II	2018	CD34 and/or CD133 stem cells	Yes, ex vivo	Lentivirus	Autologous	Inflammatory and immune system	X-Linked SCID
Cancer Research UK	A Cancer Research UK Phase I/II Trial of AST-VAC2 (Allogeneic Dendritic Cell Vaccine) Administered Weekly Via Intradermal Injection in Patients with Non-small Cell Lung Cancer (NSCLC) in the Advanced and Adjuvant Settings	NCT03371485	Birmingham University Hospital Southampton General Hospital Queen Elizabeth Hospital Birmingham	Recruiting	Phase I/II	2018	Antigen presenting cells	No	N/A	Allogeneic	Oncology (Other)	Non-Small Cell Lung Cancer
Celgene Corp	A Global Randomized Multicenter Phase III Trial of JCAR017 Compared to Standard of Care in Adult Subjects with High-risk, Second-line, Transplant-eligible Relapsed or Refractory Aggressive B-cell Non-Hodgkin Lymphomas (TRANSFORM)	NCT03575351 2018-000929-32	UCL Cancer Institute London University Hospital Southampton NHS Foundation Trust	Recruiting	Phase III	2018	T cells	Yes, ex vivo	Lentivirus	Autologous	Oncology (Haematology)	B-cell Non-Hodgkin Lymphomas
Celgene Corp	A Phase II, Single-arm, Multi-center Trial to Determine the Efficacy and Safety of JCAR017 in Subjects with Relapsed or Refractory Diffuse Large B-cell Lymphoma or with Other Aggressive B-cell Malignancies	NCT03484702 2017-000106-38	UCL Cancer Institute, London The Christie NHS Foundation Trust, Manchester	Recruiting	Phase II	2018	T cells	Yes, ex vivo	Lentivirus	Autologous	Oncology (Haematology)	B-Cell Non-Hodgkin Lymphoma
Celixir Ltd	A Phase IIB, Randomized, Double-blinded, Placebo-controlled Study of the Efficacy and Safety of Intramyocardial Injection of Allogeneic Human iMP Cells in Patients Undergoing CABG Surgery	NCT03515291 2017-000516-42	Sites not specified	Terminated	Phase II	2018	Other	No	N/A	Allogeneic	Cardiovascular	Ischaemic heart disease post Myocardial Infarction

Sponsor	Title	Clinical Database Numbers	United Kingdom Site(s)	Trial Status	Trial Phase	Year Trial Started	Cell Type	Gene Modification/ Gene Therapy	Type of Virus Vector Used (if applicable)	Autologous/ Allogeneic	Disease Area	Indication
Celyad Oncology	An Open-label, Phase I Study to Assess the Safety, Cell Kinetics and Clinical Activity of Multiple Doses of CYAD-101, Administered Concurrently with the FOLFOX Treatment in Subjects with Unresectable Metastatic Colorectal Cancer	NCT03692429	Guy's and St Thomas' NHS Foundation Trust, London The Christie NHS Foundation Trust, Manchester	Withdrawn	Phase I	2018	T cells	Yes, ex vivo	Retrovirus	Allogeneic	Oncology (Other)	Metastatic Colorectal Cancer
Cook MyoSite Inc	CELLEBRATE: An Adaptive, Two-Stage, Double-blind, Stratified, Randomized, Controlled Trial Comparing the Safety and Efficacy of AMDC-USR With Placebo in Female Subjects With Stress Urinary Incontinence	NCT03104517 2017-000956-25	Derriford Hospital, Plymouth St. James's University Hospital, Leeds	Recruiting	Phase III	2018	Skeletal Muscle	No	N/A	Autologous	Renal and Urogenital	Stress Urinary Incontinence
Freeline Therapeutics Ltd	An Open-label, Multicentre, Long-term Follow-up Study to Investigate the Safety and Durability of Response Following Dosing of a Novel Adeno-associated Viral Vector (FLT180a) in Patients with Haemophilia B	NCT03641703 2017-005080-40	Royal Free London NHS Foundation Trust Oxford University Hospitals NHS Foundation Trust	Recruiting	Phase II/III	2018	N/A	Yes, in vivo	AAV	N/A	Haematological	Haemophilia B
Gamida Cell Ltd	A Multicenter, Randomized, Phase III Registration Trial of Transplantation of Nicord, Ex Vivo Expanded, Ucb-derived, Stem and Progenitor Cells, Vs. Unmanipulated UCB for Patients with Hematological Malignancies	NCT02730299 2016-000704-28	The Royal Marsden NHS Foundation Trust, London Queen Elizabeth Hospital, Birmingham St James Hospital, Leeds Manchester Royal Infirmary Royal Manchester Children's Hospital	In follow-up	Phase III	2018	CD34 and/or CD133 stem cells	No	N/A	Allogeneic	Oncology (Haematology)	Leukemia, Lymphoma, and Myelodysplastic Syndrome (MDS)
GenSight Biologics SA	Long-term Follow-up of ND4 LHON Subjects Treated with GS010 Ocular Gene Therapy in the Rescue or Reverse Phase III Clinical Trials	NCT03406104 2017-002153-11	Moorfields Eye Hospital NHS Foundation Trust London	In follow-up	Phase III	2018	N/A	Yes, in vivo	AAV	N/A	Ophthalmology	Leber Hereditary Optic Neuropathy
GenSight Biologics SA	Efficacy and Safety of Bilateral Intravitreal Injection of GS010: A Randomized, Double-masked, Placebo-controlled Trial in Subjects Affected with G11778A ND4 Leber Hereditary Optic Neuropathy for up to One Year	NCT03293524 2017-002187-40	Moorfields Eye Hospital NHS Foundation Trust London	In follow-up	Phase III	2018	N/A	Yes, in vivo	AAV	N/A	Ophthalmology	Leber Hereditary Optic Neuropathy
Gilead Sciences Inc	A Phase III, Randomized, Open-label Study Evaluating Efficacy of Axicabtagene Ciloleucef Versus Standard of Care Therapy in Subjects with Relapsed/Refractory Diffuse Large B Cell Lymphoma	NCT03391466 2017-002261-22	University College London Hospitals The Christie NHS Foundation Trust, Manchester Barts Health NHS Trust, London University Hospitals Birmingham NHS Foundation Trust The Royal Marsden NHS Foundation Trust, Sutton	In follow-up	Phase III	2018	T cells	Yes, ex vivo	Retrovirus	Autologous	Oncology (Haematology)	B-Cell Lymphoma
Great Ormond Street Hospital for Children NHS Trust	Phase I/II Study of Lentiviral Gene Transfer for SCID-X1 With Low Dose Targeted Busulfan	NCT03601286 2018-000673-68	Great Ormond Street Hospital for Children NHS Foundation Trust, London	Recruiting	Phase I/II	2018	CD34 and/or CD133 stem cells	Yes, ex vivo	Lentivirus	Autologous	Inflammatory and immune system	X-Linked Severe Combined Immune Deficiency (SCID)
Gyroscope Therapeutics Ltd	An Open Label First in Human Phase I/II Multicentre Study to Evaluate the Safety, Dose Response and Efficacy of GT005 Administered as a Single Subretinal Injection in Subjects with Macular Atrophy Due to AMD (FocusS)	NCT03846193 2017-003712-39	Nottingham University Hospital Southampton General Hospital London Vision Clinic Moorfields Eye Hospital, London Sunderland Eye Infirmary Manchester Eye Hospital Bristol Eye Hospital Oxford University Hospital	Recruiting	Phase I/II	2018	N/A	Yes, in vivo	AAV	N/A	Ophthalmology	Age-related Macular Degeneration (AMD)
Holostem Therapie Avanzate SRL	Multinational, Multicenter, Prospective, Long-term Safety and Efficacy Follow-up Study After Autologous Cultivated Limbal Stem Cells Transplantation (ACLSTCT) for Restoration of Corneal Epithelium in Patients With Limbal Stem Cell Deficiency Due to Ocular Burns	NCT03288844 2015-001344-11	Newcastle upon Tyne Hospitals NHS Foundation Trust	Completed	Phase IV	2018	Corneal	No	N/A	Autologous	Ophthalmology	Burns Unspecified Ophthalmological Disorders
MeiraGTx Holdings Plc	Long Term Follow-Up Study of Participants Following an Open Label, Multi-Centre, Phase I/II Dose Escalation Trial of a Recombinant Adeno-Associated Virus Vector (AAV2-RPGR) for Gene Therapy of Adults and Children With X-Linked Retinitis Pigmentosa Owing to Defects in Retinitis Pigmentosa GTPase Regulator (RPGR)	NCT04312672 2018-000425-31	Moorfields Eye Hospital, London	Recruiting	Phase I/II	2018	N/A	Yes, in vivo	AAV	N/A	Ophthalmology	X-Linked Retinitis Pigmentosa
Neurotech Pharmaceuticals Inc	A Phase III Multicenter Randomized, Sham Controlled, Study to Determine the Safety and Efficacy of Renuxus in Macular Telangiectasia Type 2	NCT03316300 2017-003234-82	Oxford Eye Hospital Moorfields Eye Hospital London	In follow-up	Phase III	2018	Retinal	Yes, ex vivo	Plasmid	Allogeneic	Ophthalmology	Macular Telangiectasia
Novartis AG	A Phase II, Single Arm, Multicenter Open Label Trial to Determine the Efficacy and Safety of Tisagenlecleucel (CTL019) in Adult Patients with Refractory or Relapsed Follicular Lymphoma	NCT03568461 2017-004385-94	Queen Elizabeth Hospital, Birmingham Kings College Hospital, London	Recruiting	Phase II	2018	T cells	Yes, ex vivo	Lentivirus	Autologous	Oncology (Haematology)	Follicular Lymphoma

Sponsor	Title	Clinical Database Numbers	United Kingdom Site(s)	Trial Status	Trial Phase	Year Trial Started	Cell Type	Gene Modification/ Gene Therapy	Type of Virus Vector Used (if applicable)	Autologous/ Allogeneic	Disease Area	Indication
Orbsen Therapeutics Ltd	Repair of Acute Respiratory Distress Syndrome by Stromal Cell Administration (REALIST): An Open Label Dose Escalation Phase I Trial Followed by a Randomized, Double-blind, Placebo-controlled Phase II Trial	NCT03042143 2017-000584-33	Belfast Health and Social Care Trust, Northern Ireland Queen Elizabeth Hospital NHS Foundation Trust Manchester Royal Infirmary Wythenshawe, Trafford, Withington and Altrincham Hospitals Edinburgh Royal Infirmary Cambridge University Hospitals NHS Foundation Trust	Recruiting	Phase I/II	2018	Mesenchymal stem/stromal cells	No	N/A	Allogeneic	Respiratory	Acute Respiratory Distress Syndrome (ARDS)
RHEACELL GmbH & Co KG	An Interventional, Multicenter, Single Arm, Phase I/IIa Clinical Trial to Investigate the Efficacy and Safety of Allo-APZ2-EB on Epidermolysis Bullosa (EB)	NCT03529877 2018-001009-98	Great Ormond Street Hospital, London King's College London; St John's Institute of Dermatology	Recruiting	Phase I/II	2018	Mesenchymal stem/stromal cells	No	N/A	Allogeneic	Dermatological	Epidermolysis Bullosa (EB)
Sangamo Therapeutics Inc	A Phase I/II, Open-Label, Ascending Dose Study to Assess the Safety and Tolerability of AAV2/6 Factor IX Gene Therapy Via Zinc Finger Nuclease (ZFN) Mediated Targeted Integration of SB-FIX in Adult Subjects with Severe Hemophilia B	NCT02695160 2017-004805-42	Hammersmith Hospital, London Guy's and St Thomas' NHS Foundation Trust, London Queen Elizabeth Hospital, Birmingham Glasgow Royal Infirmary	Completed	Phase I	2018	N/A	Yes, in vivo	AAV	N/A	Haematological	Hemophilia B (Factor IX Deficiency) Mucopolysaccharidosis I (MPS I) (Hurler Syndrome)
Sangamo Therapeutics Inc	A Phase I/II, Multicenter, Open-label, Single-dose, Dose-ranging Study to Assess the Safety and Tolerability of SB-913, a rAAV2/6-based Gene Transfer in Subjects with Mucopolysaccharidosis II (MPS II)	NCT03041324 2018-000192-33	3 sites (not specified) Royal Manchester Children's Hospital	Completed	Phase I/II	2018	N/A	Yes, in vivo	AAV	N/A	Metabolic (Other)	Mucopolysaccharidosis II (MPS II) (Hunter Syndrome) - Lysosome storage disorder
Sangamo Therapeutics Inc	A Phase I/II, Multicenter, Open-label, Single-dose, Dose-ranging Study to Assess the Safety and Tolerability of SB-318, a rAAV2/6-based Gene Transfer in Subjects with Mucopolysaccharidosis I (MPS I)	NCT02702115 2018-000206-28	Sites not specified	Completed	Phase I/II	2018	N/A	Yes, in vivo	AAV	N/A	Metabolic (Other)	Adenosine Deaminase Deficiency
Takeda Pharmaceutical Co Ltd	An Observational Post-marketing Registry on the Effectiveness and Safety of Darvadstrocel in Patients with Crohn's Disease and Complex Perianal Fistulas (INSPIRE)	Alofisel-5003 EUPAS24267	St Mark's Hospital Northern General Hospital	Recruiting	Phase IV	2018	Mesenchymal stem/stromal cells	No	N/A	Allogeneic	Gastrointestinal (Other)	Crohn's Disease (Regional Enteritis), Anal Fistula
Ultragenyx Pharmaceutical Inc	A Long-term Follow-up Study to Evaluate Safety and Efficacy of Adeno-associated Virus (AAV) Serotype 8 (AAV8)-mediated Gene Transfer of Human Ornithine Transcarbamylase (OTC) in Adults with Late-onset OTC Deficiency	NCT03636438 2018-000156-18	Queen Elizabeth Hospital, Birmingham	In follow-up	Phase I/II	2018	N/A	Yes, in vivo	AAV	N/A	Metabolic (Other)	Ornithine-Transcarbamylase Deficiency
UniQure NV	Phase III, Open-label, Single-dose, Multi-center, Multinational Trial Investigating a Serotype 5 Adeno-associated Viral Vector Containing the Padua Variant of a Codon-optimized Human Factor IX Gene (AAV5-hFIXco-Padua, AMT-061) Administered to Adult Subjects with Severe or Moderately Severe Hemophilia B	NCT03569891 2017-004305-40	University Hospitals Bristol NHS Foundation Trust Addenbrooke's Hospital, Cambridge The Royal London Hospital University Hospital Southampton NHS Foundation Trust	In follow-up	Phase III	2018	N/A	Yes, in vivo	AAV	N/A	Haematological	Haemophilia B
University of Birmingham	An Adaptive, Multicentre, Phase I/IIa, Multi-disease Trial Investigating the Safety and Activity of a Single Infusion of Selected Mesenchymal Stromal Cells in the Treatment of Patients with Primary Sclerosing Cholangitis and Autoimmune Hepatitis	NCT02997878	Queen Elizabeth Hospital, Birmingham Nottingham University Hospital John Radcliffe Hospital, Oxford	Recruiting	Phase I/II	2018	Mesenchymal stem/stromal cells	No	N/A	Allogeneic	Gastrointestinal (Other)	Primary Sclerosing Cholangitis
University of Oxford	The TWO study: Transplantation without Over-immunosuppression - A Phase Ib Trial of Regulatory T Cells in Renal Transplantation	ISRCTN11038572 2017-001421-41	Churchill Hospital, Oxford	Recruiting	Phase II	2018	T cells	No	N/A	Autologous	Inflammatory and immune system	Kidney Transplant Rejection
Vertex Pharmaceuticals Inc	A Phase I/II Study to Evaluate the Safety and Efficacy of a Single Dose of Autologous CRISPR-Cas9 Modified CD34+ Human Hematopoietic Stem and Progenitor Cells (CTX001) in Subjects with Severe Sickle Cell Disease	NCT03745287 2018-001320-19	Imperial College Healthcare NHS Trust, Hammersmith Hospital	Recruiting	Phase I/II	2018	CD34 and/or CD133 stem cells	Yes, ex vivo	Non-viral	Autologous	Haematological	Severe Sickle Cell Disease (SCD)
Vertex Pharmaceuticals Inc	A Phase I/II Study of the Safety and Efficacy of a Single Dose of Autologous CRISPR-Cas9 Modified CD34+ Human Hematopoietic Stem and Progenitor Cells (hHSPCs) in Subjects with Transfusion-dependent β-thalassaemia	NCT03655678 2017-003351-38	Imperial College Healthcare, London	Completed	Phase I/II	2018	CD34 and/or CD133 stem cells	Yes, ex vivo	Non-viral	Autologous	Haematological	Transfusion-Dependent β Thalassemia
Abeona Therapeutics Inc	Phase I/II Gene Transfer Clinical Trial of rAAV9. CMV.hNAGLU for Mucopolysaccharidosis (MPS) IIIB	2014-001411-39	Sites not specified	Withdrawn	Phase I/II	2019	N/A	Yes, in vivo	AAV	N/A	Neurological	Mucopolysaccharidosis III (MPS III) (Sanfilippo Syndrome)
Achilles Therapeutics Ltd	An Open-label, Multi-Centre Phase I/IIa Study Evaluating the Safety and Clinical Activity of Neoeantigen Reactive T Cells in Patients with Metastatic or Recurrent Melanoma	NCT03997474 2018-003446-16	The Christie NHS Foundation Trust, Manchester Freeman Hospital, Newcastle upon Tyne Cambridge University Hospitals NHS Foundation Trust	Recruiting	Phase I/II	2019	T cells	No	N/A	Autologous	Oncology (Other)	Metastatic or Recurrent Melanoma

Sponsor	Title	Clinical Database Numbers	United Kingdom Site(s)	Trial Status	Trial Phase	Year Trial Started	Cell Type	Gene Modification/ Gene Therapy	Type of Virus Vector Used (if applicable)	Autologous/ Allogeneic	Disease Area	Indication
Achilles Therapeutics Ltd	An Open-Label, Multi-Centre Phase I/IIa Study Evaluating the Safety and Clinical Activity of Neoeantigen Reactive T Cells in Patients with Advanced Non-Small Cell Lung Cancer	NCT04032847 2018-001005-85	Queen Elizabeth Hospital, Birmingham Cambridge University Hospitals NHS Foundation Trust The Christie NHS Foundation Trust, Manchester University College London Hospitals University Hospital Southampton NHS Foundation Trust Freeman Hospital, Newcastle upon Tyne	Recruiting	Phase I/II	2019	T cells	No	N/A	Autologous	Oncology (Other)	Non-Small Cell Lung Cancer
BioMarin Pharmaceutical Inc	A Phase I/II Open-Label, Dose Escalation Study to Determine the Safety and Efficacy of BMN 307, an Adeno-Associated Virus Vector-Mediated Gene Transfer of Human Phenylalanine Hydroxylase in Subjects With Phenylketonuria	NCT04480567 2019-001878-28	University Hospitals Birmingham NHS Foundation Trust	Suspended	Phase I/II	2019	N/A	Yes, in vivo	AAV	N/A	Metabolic (Other)	Phenylketonuria (PKU)
bluebird bio Inc	A Phase II/III Study of Lenti-D Drug Product After Myeloablative Conditioning Using Busulfan and Fludarabine in Subjects <=17 Years of Age with Cerebral Adrenoleukodystrophy (CALD)	NCT03852498 2018-001145-14	UCL Great Ormond Street Institute of Child Health	Recruiting	Phase II/III	2019	CD34 and/or CD133 stem cells	Yes, ex vivo	Lentivirus	Autologous	Metabolic (Other)	Adrenoleukodystrophy
Celgene Corp	A Phase III, Multicenter, Randomized, Open-label Study to Compare the Efficacy and Safety of Bb2121 Versus Standard Regimens in Subjects with Relapsed and Refractory Multiple Myeloma (RRMM) (KarMMA-3)	NCT03651128 2018-001023-38	2 sites (not specified)	In follow-up	Phase III	2019	T cells	Yes, ex vivo	Lentivirus	Autologous	Oncology (Haematology)	Multiple Myeloma
Celgene Corp	Long-Term Follow-up Protocol for Subjects Treated With Gene-Modified T Cells	NCT03435796 2017-001465-24	Somers Cancer Research Building, Southampton Christie Hospital NHS Trust, Manchester Kings College Hospital, London UCL Cancer Institute, London Leeds Teaching Hospitals NHS Foundation Trust	Recruiting	Phase II/III	2019	T cells	Yes, ex vivo	Lentivirus	Autologous	Oncology (Other)	Oncology
Celgene Corp	A Phase II, Multicohort, Open-label, Multicenter Study to Evaluate the Efficacy and Safety of bb2121 in Subjects With Relapsed and Refractory Multiple Myeloma and in Subjects with Clinical High-Risk Multiple Myeloma (KarMMA-2)	NCT03601078 2018-000264-28	King's College Hospital, London	In follow-up	Phase II	2019	T cells	Yes, ex vivo	Lentivirus	Autologous	Oncology (Other)	Multiple Myeloma
Collectis SA	Phase I, Open Label Dose-escalation Study to Evaluate the Safety, Expansion, Persistence and Clinical Activity of Multiple Infusions of Ucart123 (Allogeneic Engineered T-cells Expressing Anti-cd123 Chimeric Antigen Receptor) in Patients with Adverse Genetic Risk Acute Myeloid Leukaemia	NCT04106076	King's College Hospital NHS Foundation Trust	Withdrawn	Phase I	2019	T cells	Yes, ex vivo	Lentivirus	Allogeneic	Oncology (Haematology)	Acute Myelocytic Leukemia (AML, Acute Myeloblastic Leukemia)
Freeline Therapeutics Ltd	A Phase I/II, Baseline-controlled, Non-randomised, Open-label, Single-ascending Dose Study of a Novel Adeno-associated Viral Vector (Fit190) in Patients with Fabry Disease	NCT04040049 2018-002097-51	Royal Free Hospital, London	Recruiting	Phase I/II	2019	N/A	Yes, in vivo	AAV	N/A	Metabolic (Other)	Fabry Disease - a Lysosomal Storage Disorder
GlaxoSmithKline Plc	Master Protocol to Assess the Safety and Antitumor Activity of Genetically Engineered NY-ESO-1-Specific (c259) T Cells, Alone or in Combination With Other Agents, in HLA-A2+ Participants With NY-ESO-1 and/or LAGE-1a Positive Solid Tumors (IGNYTE-ESO)	NCT03967223 2019-000415-87	GSK Investigational Site (not specified), London The Christie NHS Foundation Trust, Manchester	Recruiting	Phase II	2019	T cells	Yes, ex vivo	Lentivirus	Autologous	Oncology (Other)	Synovial Sarcoma Solid Tumor
GlaxoSmithKline Plc	A Phase Ib/IIa Pilot Study to Evaluate the Safety and Tolerability of Autologous T-Cells Expressing Enhanced TCRs (T Cell Receptors) Specific for NY-ESO-1/LAGE-1a (GSK337794) Alone, or in Combination With Pembrolizumab in HLA-A2+ Participants With NY-ESO-1- or LAGE-1a-Positive Advanced or Recurrent Non-Small Cell Lung Cancer	NCT03709706 2018-003949-42	University College London Hospitals The Christie NHS Foundation Trust, Manchester	Recruiting	Phase I/II	2019	T cells	Yes, ex vivo	Lentivirus	Autologous	Oncology (Other)	Non-Small Cell Lung Cancer
Iovance Biotherapeutics Inc	A Phase II, Multicenter Study of Autologous Tumor Infiltrating Lymphocytes (LN 144/LN-145/ LN-145-S1) in Patients with Solid Tumors	NCT03645928 2018-001608-12	Guy's Hospital, London Bristol Haematology and Oncology Centre	Recruiting	Phase II	2019	T cells	No	N/A	Autologous	Oncology (Other)	Metastatic Melanoma
Kite, A Gilead Company	A Phase I Multicenter Study Evaluating the Safety and Tolerability of KTE-X19 in Adult Subjects with Relapsed/Refractory Chronic Lymphocytic Leukemia and Small Lymphocytic Lymphoma	NCT03624036 2018-001923-38	Cambridge University Hospitals NHS Foundation Trust	Withdrawn	Phase I	2019	T cells	Yes, ex vivo	Retrovirus	Autologous	Oncology (Other)	Refractory Chronic Lymphocytic Leukemia (CLL)

Sponsor	Title	Clinical Database Numbers	United Kingdom Site(s)	Trial Status	Trial Phase	Year Trial Started	Cell Type	Gene Modification/ Gene Therapy	Type of Virus Vector Used (if applicable)	Autologous/ Allogeneic	Disease Area	Indication
Kuopio University Hospital	Clinical Development and Proof of Principle Testing of New Regenerative Adenovirus Vascular Endothelial Growth Factor (VEGF-D) Therapy for Cost-effective Treatment of Refractory Angina. A Phase II Randomized, Double-blinded, Placebo-controlled Study (ReGenHeart)	NCT03039751 2017-000789-31	Queen Mary University of London	In planning/ set-up	Phase II	2019	N/A	Yes, in vivo	Adenovirus	N/A	Cardiovascular	Angina (Angina Pectoris) Coronary Artery Disease (CAD) (Ischemic Heart Disease) Refractory Angina
Lysogene SAS	Open-label, Single-arm, Multi-center Study of Intracerebral Administration of Adeno-associated Viral (AAV) Serotype rh.10 Carrying Human N-sulfoglucosaminyl Sulfohydrolase (SGSH) cDNA for Treatment of Mucopolysaccharidosis Type IIIA	NCT03612869 2018-000195-15	Great Ormond Street Hospital, London	In follow-up	Phase III/III	2019	N/A	Yes, in vivo	AAV	N/A	Neurological	Mucopolysaccharidosis Type IIIA
MeiraGTx Holdings Plc	An Open Label, Multi-centre, Phase I/II Dose Escalation Trial of a Recombinant Adeno-associated Virus Vector (AAV2/8-hG1.7p-coCNGA3) for Gene Therapy of Children with Achromatopsia Owing to Defects in CNGA3	NCT03758404 2018-003431-29	Moorfields Eye Hospital, London	Recruiting	Phase I/II	2019	N/A	Yes, in vivo	AAV	N/A	Ophthalmology	Achromatopsia
Novartis AG	Tisagenlecleucel Versus Standard of Care in Adult Patients with Relapsed or Refractory Aggressive B-cell Non-hodgkin Lymphoma: A Randomized, Open Label, Phase III Trial (BELINDA)	NCT03570892 2016-002966-29	Queen Elizabeth Hospital, Birmingham University College London Hospitals	Recruiting	Phase III	2019	T cells	Yes, ex vivo	Lentivirus	Autologous	Oncology (Haematology)	Non-Hodgkin Lymphoma
Novartis AG	A Phase II, Single Arm, Multicenter Open Label Trial to Determine the Safety and Efficacy of Tisagenlecleucel in Pediatric Subjects With Relapsed or Refractory Mature B-cell Non-Hodgkin Lymphoma (NHL) (BIANCA)	NCT03610724 2017-005019-15	2 sites (not specified) Royal Manchester Children's Hospital	Recruiting	Phase II	2019	T cells	Yes, ex vivo	Lentivirus	Autologous	Oncology (Haematology)	B-Cell Non-Hodgkin Lymphoma
Novartis AG	A Phase II Trial of Tisagenlecleucel in First-line High-risk (HR) Pediatric and Young Adult Patients with B-cell Acute Lymphoblastic Leukemia (B-ALL) Who Are Minimal Residual Disease (MRD) Positive at the End of Consolidation (EOC) Therapy	NCT03876769 2017-002116-14	Great Ormond Street Hospital, London University College London Hospitals	Recruiting	Phase II	2019	T cells	Yes, ex vivo	Lentivirus	Autologous	Oncology (Haematology)	ALL, B-cell Leukaemia
Oragenics Inc	A Phase IIb, Multi-center, Randomized, Double-blind, Placebo-controlled Study to Assess the Safety and Efficacy of Topically-applied AG013 for the Attenuation of Oral Mucositis in Subjects with Cancers of the Head and Neck Receiving Concomitant Chemoradiation Therapy	NCT03234465 2016-004161-68	Queen Elizabeth Hospital, Birmingham Beatson West of Scotland Cancer Centre, Glasgow Royal Cornwall Hospital, Truro Derriford Hospital, Plymouth The Christie NHS Foundation Trust, Manchester Guy's Hospital, London	Terminated	Phase II	2019	Other	Yes, ex vivo	N/A	N/A	Oral	Oral Mucositis (OM)
Orchard Therapeutics Plc	An Observational Long-term Follow-up Study for Patients Previously Treated with Autologous ex Vivo Gene Therapy for Severe Combined Immunodeficiency Due to Adenosine Deaminase Deficiency (ADA-SCID)	NCT04049084	UCL Great Ormond Street Institute of Child Health, London	Recruiting	Phase III	2019	CD34 and/or CD133 stem cells	Yes, ex vivo	Lentivirus	Autologous	Inflammatory and immune system	Adenosine Deaminase (ADA) Deficiency Related SCID
Pluristem Therapeutics Inc	Phase III, Multicenter, Randomized, Double-Blind, Placebo-Controlled Study, Designed to Determine the Efficacy, Safety, and Tolerability of Intramuscular Administration of Allogeneic PLX-PAD Cells for the Treatment of Muscle Injury Following Arthroplasty for Hip Fracture	NCT03451916 2017-005165-49	Norfolk and Norwich University Hospital John Radcliffe Hospital Oxford	Recruiting	Phase III	2019	Mesenchymal stem/stromal cells	No	N/A	Allogeneic	Musculoskeletal	Bone Fracture Muscle Injury
RHEACELL GmbH & Co KG	A Randomised, Placebo-controlled, Double-blind, Interventional, Multicenter, Phase I/IIa Clinical Trial to Investigate the Efficacy and Safety of Allo-AP22-PAOD for the Treatment of Peripheral Arterial Occlusive Disease (PAOD)	NCT03339973 2017-000235-14	East Surrey Hospital	Terminated	Phase I/II	2019	Mesenchymal stem/stromal cells	No	N/A	Allogeneic	Cardiovascular	Peripheral Artery Occlusive Disease (PAOD)
Sangamo Therapeutics Inc	A Phase I/II, Multicenter, Open-label, Single-dose, Dose-ranging Study to Assess the Safety and Tolerability of ST-920, a rAAV2/6 Human Alpha Galactosidase A Gene Therapy, in Subjects with Fabry Disease	NCT04046224 2019-000667-24	Queen Elizabeth Hospital NHS Foundation Trust Cambridge University Hospitals NHS Foundation Trust	In planning/ set-up	Phase I/II	2019	N/A	Yes, in vivo	AAV	N/A	Metabolic (Other)	Fabry Disease
Solid Biosciences Inc	A Randomized, Controlled, Open-label, Single-ascending Dose, Phase I/II Study to Investigate the Safety and Tolerability, and Efficacy of Intravenous SGT-001 in Male Adolescents and Children with Duchenne Muscular Dystrophy	NCT03368742 2017-002213-60	Sites not specified	Withdrawn	Phase I/II	2019	N/A	Yes, in vivo	AAV	N/A	Musculoskeletal	Duchenne Muscular Dystrophy
The University of Manchester	Mesoangioblast-Mediated Exon 51 Skipping For Genetic Correction Of Dystrophin, Based Upon A Single Injection In Individual Skeletal Muscles Of Five Non Ambulant Patients Affected By Duchenne Muscular Dystrophy: A Non Randomized, Open Label, Phase I/IIa Study.	2019-001825-28	Royal Manchester Children's Hospital	In planning/ set-up	Phase I/II	2019	Other	Yes, ex vivo	Lentivirus	Autologous	Musculoskeletal	Duchenne Muscular Dystrophy

Sponsor	Title	Clinical Database Numbers	United Kingdom Site(s)	Trial Status	Trial Phase	Year Trial Started	Cell Type	Gene Modification/ Gene Therapy	Type of Virus Vector Used (if applicable)	Autologous/ Allogeneic	Disease Area	Indication
TiGenix NV	A Phase-III Randomized, Double-blind, Parallel-group, Placebo-controlled, International, Multicentre Study to Assess Efficacy and Safety of Cx601, Adult Allogeneic Expanded Adipose-derived Stem Cells (eASC) for the Treatment of Complex Perianal Fistula(s) in Patients With Crohn's Disease Over a Period of 24 Weeks and a Follow up Period up to 52 Weeks	NCT03279081 2017-000725-12	Glasgow Royal Infirmary Addenbrooke's Hospital, Cambridge Royal Devon And Exeter Hospital, Exeter Wythenshawe Hospital, Manchester St. Mark's Hospital, London St Thomas' Hospital, London Nottingham University Hospitals NHS Trust, Nottingham Northern General Hospital, Sheffield	Recruiting	Phase III	2019	Adipose-derived stem cells	No	N/A	Allogeneic	Gastrointestinal (Other)	Crohn's Disease
Trizell Ltd	A Phase III, Open-label, Randomized, Parallel Group Study to Evaluate the Efficacy and Safety of Intrapleural Administration of Adenovirus-delivered Interferon Alpha-2b (rAd-IFN) in Combination with Celecoxib and Gemcitabine in Patients with Malignant Pleural Mesothelioma	NCT03710876 2017-003169-82	Beatson West of Scotland Cancer Centre, Glasgow Derriford Hospital, Plymouth Royal Marsden Foundation Trust, London Guy's and St. Thomas' NHS Trust, London Wythenshawe Hospital, Manchester Churchill Hospital, Oxford	Recruiting	Phase III	2019	N/A	Yes, in vivo	Adenovirus	N/A	Oncology (Other)	Malignant Pleural Mesothelioma
University College London	Targeted Stem Cells Expressing Trail as a Therapy for Lung Cancer	NCT03298763	University College London Hospitals	Recruiting	Phase I/II	2019	Mesenchymal stem/stromal cells	Yes, ex vivo	Lentivirus	Allogeneic	Oncology (Other)	Non-Small Cell Lung Cancer
University College London	Phase I Study of Transfer of Effector Memory T Cells (Tem) Following Allogeneic Stem Cell Transplantation	NCT03836690	University College London Hospitals	Recruiting	Phase I	2019	T cells	No	N/A	Allogeneic	Oncology (Haematology)	Lymphoma Leukemia Myelodysplastic Syndrome Aplastic Anemia Primary Immune Deficiency (PID) Graft Versus Host Disease (GVHD) Non-Hodgkin Lymphoma Hodgkin Lymphoma (B-Cell Hodgkin Lymphoma) Chronic Lymphocytic Leukemia (CLL) Acute Myelocytic Leukemia (AML, Acute Myeloblastic Leukemia) Acute Lymphocytic Leukemia (ALL, Acute Lymphoblastic Leukemia) Multiple Myeloma (Kahler Disease) Chronic Myelomonocytic Leukemia (CMML)
University College London	Immunotherapy with Tacrolimus Resistant EBV CTL for Lymphoproliferative Disease after Solid Organ Transplant	NCT03131934	Great Ormond Street Hospital, London King's College Hospital, London	Recruiting	Phase I	2019	T cells	Yes, ex vivo	Retrovirus	Autologous	Oncology (Haematology)	Post-Transplant Lymphoproliferative Disorder
Adaptimmune Therapeutics Plc	A Phase II Single Arm Open-label Clinical Trial of ADP-A2M4 Spear T Cells in Subjects with Advanced Synovial Sarcoma or Myxoid/Round Cell Liposarcoma	NCT04044768 2019-000589-39	University College London Hospitals The Christie NHS Foundation Trust, Manchester	Recruiting	Phase II	2020	T cells	Yes, ex vivo	Lentivirus	Autologous	Oncology (Other)	Synovial Sarcoma, Myxoid/round cell liposarcoma
Atara Biotherapeutics Inc	Multi-center, Open Label, Phase III Study of Tabelecleucel for Solid Organ or Allogeneic Hematopoietic Cell Transplant Subjects With Epstein-Barr Virus-Associated Post-Transplant Lymphoproliferative Disease After Failure of Rituximab or Rituximab and Chemotherapy	NCT03394365 2017-002949-30	Cambridge University Hospitals NHS Foundation Trust	In planning/ set-up	Phase III	2020	T cells	No	N/A	Allogeneic	Oncology (Other)	Epstein-Barr Virus-Associated Post-Transplant Lymphoproliferative Disease
Audentes Therapeutics Inc	A Phase I/II, Open-label, Ascending-dose Clinical Study to Evaluate the Safety and Preliminary Efficacy of AT845, an AAV8-delivered Gene Transfer Therapy in Patients with Late Onset Pompe Disease	NCT04174105	The Newcastle upon Tyne Hospitals NHS Foundation Trust	In planning/ set-up	Phase I/II	2020	N/A	Yes, in vivo	AAV	N/A	Metabolic (Other)	Pompe Disease
Autolus Therapeutics Plc	An Open-label, Multi-centre, Phase Ib/II Study Evaluating the Safety and Efficacy of AUTO1, a CAR T Cell Treatment Targeting CD19, in Adult Patients with Relapsed or Refractory B Cell Acute Lymphoblastic Leukaemia	NCT04404660 2019-001937-16	University Hospitals Bristol and Weston NHS Foundation Trust Queen Elizabeth Hospital NHS Foundation Trust University College London Hospitals NHS Foundation Trust King's College Hospital, London Manchester Royal Infirmary Hospital Freeman Hospital, Newcastle upon Tyne Beatson West of Scotland Cancer Centre, Glasgow	Recruiting	Phase I/II	2020	T cells	Yes, ex vivo	Lentivirus	Autologous	Oncology (Haematology)	B-Cell Acute Lymphocytic Leukemia
AveXis, Inc.	A Long-term Follow-up Study of Patients in the Clinical Trials for Spinal Muscular Atrophy Receiving AVXS-101	NCT04042025 2019-002611-26	Great Ormond Street Hospital for Children NHS Foundation Trust Newcastle Upon Tyne Hospitals NHS Foundation Trust	Recruiting	Phase IV	2020	N/A	Yes, in vivo	AAV	N/A	Neuromuscular	Spinal Muscular Atrophy (SMA)
BioMarin Pharmaceutical Inc	A Phase I/II Safety, Tolerability, and Efficacy Study of BMN 270, an Adeno-associated Virus Vector-mediated Gene Transfer of Human Factor VIII in Hemophilia A Patients with Active or Prior Inhibitors	2019-003213-34	Queen Elizabeth Hospital NHS Foundation Trust	Recruiting	Phase I/II	2020	N/A	Yes, in vivo	AAV	N/A	Haematological	Hemophilia A (Factor VIII Deficiency)
bluebird bio Inc	Longterm Follow-up of Subjects With Hemoglobinopathies Treated With Ex Vivo Gene Therapy Using Autologous Hematopoietic Stem Cells Transduced With a Lentiviral Vector	NCT02633943 2013-002245-11	University College London Hospitals	Recruiting	Phase III	2020	CD34 and/or CD133 stem cells	Yes, ex vivo	Lentivirus	Autologous	Haematological	Sickle Cell Disease β-Thalassemia
bluebird bio Inc	A Phase II/III Study Evaluating Gene Therapy by Transplantation of Autologous CD34+ Stem Cells Transduced Ex Vivo With the LentiGloBB305 Lentiviral Vector in Subjects With Sickle Cell Disease	NCT04293185 2019-000331-63	2 sites (not specified)	In planning/ set-up	Phase III	2020	CD34 and/or CD133 stem cells	Yes, ex vivo	Lentivirus	Autologous	Haematological	Sickle Cell Disease

Sponsor	Title	Clinical Database Numbers	United Kingdom Site(s)	Trial Status	Trial Phase	Year Trial Started	Cell Type	Gene Modification/ Gene Therapy	Type of Virus Vector Used (if applicable)	Autologous/ Allogeneic	Disease Area	Indication
CCTU- Cancer Theme	A Study to Collect Bone Marrow for Process Development and Production of Bone Marrow Mesenchymal Stromal Cells to Treat Severe COVID19 Pneumonitis	NCT04397471	Cambridge University Hospitals NHS Foundation Trust	In planning/ set-up	Phase I	2020	Mesenchymal stem/stromal cells	No	N/A	Allogeneic	Respiratory	Severe COVID-19 Pneumonitis
Celgene Corp	A Phase II, Open-label, Single Arm, Multicenter Trial to Evaluate the Safety and Efficacy of JCAR017 (Lisocabtagene Maraleucl) in Adult Subjects with High-risk, Relapsed or Refractory Indolent B-cell Non-hodgkin Lymphoma (NHL)	NCT04245839 2019-004081-18	The Christie NHS Foundation Trust, Manchester University College London Hospitals	Recruiting	Phase II	2020	T cells	Yes, ex vivo	Lentivirus	Autologous	Oncology (Haematology)	Follicular Lymphoma B-Cell Non-Hodgkin Lymphoma Marginal Zone B-cell Lymphoma Nodal Marginal Zone B-Cell Lymphoma
Cell and Gene Therapy Catapult	Phase I/II, Open Label Study to Assess the Safety and Efficacy Autologous Bone Marrow-derived Mesenchymal Stromal Cells Seeded on to Decellularised Airway Scaffold in Subjects with Clinically Significant Bronchopleural Fistula	NCT04435249 2019-004939-24	Royal Papworth Hospital, Cambridge	In planning/ set-up	Phase I/II	2020	Mesenchymal stem/stromal cells	No	N/A	Autologous	Respiratory	Bronchopleural Fistula
Cynata Therapeutics Ltd	A Randomised, Double-blind, Placebo-controlled Phase II Study to Investigate the Efficacy, Safety and Tolerability of Cyp-002 in Adults with Critical Limb Ischaemia Who Are Unsuited for Revascularisation	2019-001361-33	Manchester Royal Infirmary	In planning/ set-up	Phase II	2020	Mesenchymal stem/stromal cells	No	N/A	Allogeneic	Cardiovascular	Critical limb ischemia
Freeline Therapeutics Ltd	A Multicentre, Long-term, Follow-up Study to Investigate the Safety and Durability of Response Following Dosing of an Adeno-associated Viral Vector (FLT190) in Subjects with Fabry Disease	NCT04455230 2019-004645-32	Royal Free Hospital London	Recruiting	Phase II	2020	N/A	Yes, in vivo	AAV	N/A	Metabolic (Other)	Fabry Disease Lysosomal Storage Disorders
Great Ormond Street Hospital for Children NHS Trust	Phase I, Open Label Study of CRISPR-CAR Genome Edited T Cells (TT52CAR19) in Relapsed /Refractory B Cell Acute Lymphoblastic Leukaemia	NCT04557436 2019-003462-40	Sites not specified	Recruiting	Phase I	2020	T cells	Yes, ex vivo	Lentivirus	Allogeneic	Oncology (Haematology)	B-Cell Acute Lymphocytic Leukemia (B-Cell Acute Lymphoblastic Leukaemia)
Gyroscope Therapeutics Ltd	Explore: A Phase II, Outcomes Assessor-Masked, Multicentre, Randomised Study To Evaluate The Safety and Efficacy of Two Doses of GT005 Administered As A Single Subretinal Injection In Subjects With Geographic Atrophy Secondary To Age-Related Macular Degeneration	NCT04437368 2019-003421-22	Royal Eye Hospital Manchester	In planning/ set-up	Phase II	2020	N/A	Yes, in vivo	AAV	N/A	Ophthalmology	Age-related Macular Degeneration
Gyroscope Therapeutics Ltd	HORIZON : A Phase II, Open-label, Outcomes-assessor Masked, Multicentre, Randomised, Controlled Study to Evaluate the Safety and Efficacy of Two Doses of GT005 Administered as a Single Subretinal Injection in Subjects with Geographic Atrophy Secondary to Age-related Macular Degeneration	NCT04566445 2020-002431-30	Sunderland Eye Infirmary	Recruiting	Phase II	2020	N/A	Yes, in vivo	AAV	N/A	Ophthalmology	Age Related Macular Degeneration
Hospital of the University of Munich	Treatment of Chemo-refractory Viral Infections after Allogeneicstem Cell Transplantation with Multispecific T Cells Against CMV, EBV and ADV: A Phase III, Prospective, Multicentre Clinical Trial	2018-000853-29	Newcastle Upon Tyne Hospitals NHS Foundation Trust Manchester University NHS Foundation Trust Great Ormond Street Hospital, London University College London Hospitals NHS Foundation Trust	Recruiting	Phase III	2020	T cells	No	N/A	Allogeneic	Infectious Disease	Cytomegalovirus (HHV-5) Infections Epstein-Barr Virus (HHV-4) Infections Adenoviridae Infections
Imperial College London	Pancreatic Islet Transplantation to the Anterior Chamber of the Eye	NCT04198350	Imperial College Healthcare NHS Trust, London	Suspended	Phase II	2020	Pancreatic islets	No	N/A	Autologous	Metabolic (Diabetes)	Type 1 Diabetes (Juvenile Diabetes) Unspecified Ophthalmological Disorders
Instil Bio UK Ltd	Protocol Title: An Open Label, Multi-centre Phase 1/2a Study of Modified and Unmodified Autologous Tumour Infiltrating Lymphocytes (TIL) in Patients With Platinum-resistant Ovarian Cancer	NCT04389229	Queens Elizabeth Hospital, Birmingham The Christie Hospital, Manchester	In planning/ set-up	Phase I/II	2020	T cells	Yes, ex vivo	Unknown	Autologous	Oncology (Other)	Metastatic Ovarian Cancer
Intellia Therapeutics	Phase I Two-part (Open-label, Single Ascending Dose (Part 1) and Open-label, Single Dose Expansion (Part 2)) Study to Evaluate Safety, Tolerability, Pharmacokinetics, and Pharmacodynamics of NTLA-2001 in Patients with Hereditary Transthyretin Amyloidosis with Polyneuropathy (ATTRV-PN)	NCT04601051	London site (not specified)	Recruiting	Phase I	2020	N/A	Yes, in vivo	Non-viral	N/A	Metabolic (Other)	Hereditary Transthyretin Amyloidosis With Polyneuropathy (ATTRV-PN)
Johnson & Johnson	A Phase III Randomized Study Comparing JNJ-68284528, a Chimeric Antigen Receptor T Cell (CAR-T) Therapy Directed Against BCMA, Versus Pomalidomide, Bortezomib and Dexamethasone (PvD) or Daratumumab, Pomalidomide and Dexamethasone (DPd) in Subjects with Relapsed and Lenalidomide-Refractory Multiple Myeloma	NCT04181827 2019-001413-16	Queen Elizabeth Hospital, Birmingham Bristol Royal Infirmary University College London Hospitals King's College Hospital, London Christie Hospital, Manchester Freeman Hospital, Newcastle Upon Tyne	Recruiting	Phase III	2020	T cells	Yes, ex vivo	Lentivirus	Autologous	Oncology (Haematology)	Multiple Myeloma
Novartis AG	Long Term Follow-Up of Patients Exposed to Lentiviral-Based CAR T-Cell Therapy	NCT02445222 2014-001673-14	Sites not specified	Recruiting	N/A	2020	T cells	Yes, ex vivo	Lentivirus	Autologous	Oncology (Haematology)	Unspecified B-Cell Lymphomas B-Cell Leukemia

Sponsor	Title	Clinical Database Numbers	United Kingdom Site(s)	Trial Status	Trial Phase	Year Trial Started	Cell Type	Gene Modification/ Gene Therapy	Type of Virus Vector Used (if applicable)	Autologous/ Allogeneic	Disease Area	Indication
Pfizer Inc	Phase III, Open Label, Single Arm Study to Evaluate Efficacy and Safety of Fix Gene Transfer with PF-06838435 (RAAV-SPARK100-HFIX-PADUA) in Adult Male Participants with Moderately Severe to Severe Hemophilia B (Fix:C <=2%) (BENEGENE-2)	NCT03861273 2018-003086-33	Newcastle upon Tyne Hospitals NHS Foundation Trust Glasgow Royal Infirmary	Recruiting	Phase III	2020	N/A	Yes, in vivo	AAV	N/A	Haematological	Hemophilia B (Factor IX Deficiency)
Pfizer Inc	A Phase III, Multicenter, Randomized, Double-Blind, Placebo-Controlled Study to Evaluate the Safety and Efficacy of PF-06939926 for the Treatment of Duchenne Muscular Dystrophy	NCT04281485 2019-002921-31	3 sites (not specified)	In planning/ set-up	Phase III	2020	N/A	Yes, in vivo	AAV	N/A	Musculoskeletal	Duchenne Muscular Dystrophy
Promethera Biosciences SA	Randomized, Placebo-controlled, Double Blind, Multi-centre Phase IIb Study to Evaluate the Efficacy and Safety of Hepastem in Patients with Acute on Chronic Liver Failure (ACLF)	NCT04229901 2019-003051-11	Sites not specified	Recruiting	Phase II	2020	Other	No	N/A	Allogeneic	Gastrointestinal (Liver)	Acute on Chronic Liver Failure (ACLF)
ReNeuron Group Plc	First-in-human Phase I/IIa, Open-label, Prospective Study of the Safety and Tolerability of Subretinally Transplanted Human Retinal Progenitor Cells (hRPC) in Patients with Retinitis Pigmentosa (RP)	NCT02464436 2019-004547-77	Oxford Eye Hospital, Oxford	In planning/ set-up	Phase I/II	2020	Retinal	No	N/A	Allogeneic	Ophthalmology	Retinitis Pigmentosa (Retinitis)
Rocket Pharmaceuticals	A Phase II Clinical Trial to Evaluate the Efficacy of the Infusion of Autologous CD34+ Cells Transduced With a Lentiviral Vector Carrying the FANCA Gene (Orphan Drug) in Patients With Fanconi Anemia Subtype A	NCT04069533 2018-002502-31	Great Ormond Street Hospital, London	Recruiting	Phase II	2020	CD34 and/or CD133 stem cells	Yes, ex vivo	Lentivirus	Autologous	Haematological	Fanconi Anemia
Rocket Pharmaceuticals	Gene Therapy for Leukocyte Adhesion Deficiency-i (Lad-i): A Phase I/II Clinical Trial to Evaluate the Safety and Efficacy of the Infusion of Autologous Hematopoietic Stem Cells Transduced with a Lentiviral Vector Encoding the Itgb2 Gene	NCT03812263 2020-000517-33	Great Ormond Street Hospital, London	In planning/ set-up	Phase I/II	2020	CD34 and/or CD133 stem cells	Yes, ex vivo	Lentivirus	Autologous	Haematological	Leukocyte Adhesion Deficiency-I (LAD-I)
Sangamo Therapeutics Inc	A Multicentre, Open-Label, Single Ascending Dose, Dose-Ranging, Phase I/IIa Study to Evaluate the Safety and Tolerability of an Autologous Antigen-Specific Chimeric Antigen Receptor T Regulatory Cell Therapy (TX200-TR101) in Living Donor Renal Transplant Recipients	2019-001730-34	Sites not specified	In planning/ set-up	Phase I/II	2020	T cells	Yes, ex vivo	Lentivirus	Autologous	Inflammatory and immune system	Kidney Transplant Rejection
Sigilon Therapeutics Inc	A Phase 1/2 Open-label, Dose-escalation, Safety, Tolerability, and Efficacy Study of SIG-001 in Adult Patients with Severe or Moderately-severe Haemophilia a Without Inhibitors	NCT04541628 2019-004210-33	London site (not specified) Southampton site (not specified) Manchester Royal Infirmary	Recruiting	Phase I/II	2020	Retinal	Yes, ex vivo	Non-viral	Allogeneic	Haematological	Hemophilia A (Factor VIII Deficiency)
Spark Therapeutics Inc	Phase I/II, Dose-escalation Study to Evaluate the Safety, Tolerability and Efficacy of a Single Intravenous Infusion of SPK-3006 in Adults With Late-onset Pompe Disease	NCT04093349 2019-001283-30	Salford Royal MHS Foundation Trust	Recruiting	Phase I/II	2020	N/A	Yes, in vivo	AAV	N/A	Metabolic (Other)	Lysosomal Storage Disorder
The University of Manchester Royal Manchester Children's Hospital	A Phase I/II, Study of Autologous CD34+ Haematopoietic Stem Cells Transduced ex Vivo with CD11b Lentiviral Vector Encoding for Human SGSH in Patients With Mucopolysaccharidosis Type IIIA (MPS IIIa, Sanfilippo Syndrome Type A)	NCT04201405 2019-002051-42	Central Manchester University Hospital NHS Foundation Trust	Recruiting	Phase I/II	2020	CD34 and/or CD133 stem cells	Yes, ex vivo	Lentivirus	Autologous	Metabolic (Other)	Mucopolysaccharidosis III (MPS III) (Sanfilippo Syndrome) - Lysosomal storage disease
Vascular Biogenics Ltd	A Randomized, Controlled, Double-arm, Double-blind, Multi-center Study of Ofranergene Obadenovec (Vb-111) Combined with Paclitaxel Vs. Paclitaxel Combined with Placebo for the Treatment of Recurrent Platinum-resistant Ovarian Cancer	NCT03398655 2019-003884-23	Sites not specified	In planning/ set-up	Phase III	2020	N/A	Yes, in vivo	AAV	N/A	Oncology (other)	Ovarian Cancer