Cell and Gene Therapy Catapult ATMP Clinical Trials Database 2023

Sponsor	Title	Clinical Database Numbers	Trial Phase	Trial Status	Disease Area	Indication	Cell Type	Gene Modification / Gene Therapy	Type of Vector Used (if applicable)	Autologous/ Allogeneic	Year Trial Started	United Kingdom Site(s)
2Seventy Bio Inc	A Study to Compare the Efficacy and Safety of Idecabtagene Vicleucel With Lenalidomide Maintenance Therapy Versus Lenalidomide Maintenance Therapy Alone in Adult Participants With Newly Diagnosed Multiple Myeloma Who Have Suboptimal Response After Autologous Stem Cell Transplantation	GDC30031370	Phase III	Recruiting	Oncology (Other)	Multiple Myeloma (Kahler Disease)	T cells	Yes, *ex vivo*	Lentivirus	Autologous	2023	Local Institution - 0093, Birmingham, Local Institution - 008, London, Local Institution - 0094, London, Local Institution - 008, Glasgow
Achilles Therapeutics Plc	ATL001 in Patients with Metastatic or Recurrent Melanoma	GDC30019657	Phase I/II	Recruiting	Oncology (Other)	Metastatic or Recurrent Melanoma	T cells	No	N/A	Autologous	2019	The Christie NHS Foundation Trust, Manchester Freeman Hospital, Newcastle Upon Tyne Cambridge University Hospitals NHS Foundation Trust, Cambridge University College London Hospital (UCLH), London Guy's and St Thomas' NHS Foundation Trust, London The Royal Marsden NHS Foundation Trust, London University Hospital Southampton NHS Foundation Trust, Southampton
Achilles Therapeutics Plc	ATL001 in Patients with Advanced Unresectable or Metastatic NSCLC	GDC30019861	Phase I/II	Recruiting	Oncology (Other)	Non-Small Cell Lung Cancer	T cells	No	N/A	Autologous	2021	Birmingham NHS Foundation Trust, Birmingham Cambridge University Hospitals NHS Foundation Trust, Cambridge The Christie NHS Foundation Trust, Manchester University College London Hospital (UCLH), London University Hospital Southampton NHS Foundation Trust, Southampton Freeman Hospital, Newcastle Upon Tyne The Leeds Teaching Hospitals NHS Trust, Leeds Guy's and St Thomas' NHS Foundation Trust, London
Achilles Therapeutics Plc	Long-Term Follow-Up Study of Patients Receiving ATL001	NCT04785365	Phase II	In planning/ set-up	Oncology (Other)	Metastatic or Recurrent Melanoma Non-Small Cell Lung Cancer	T cells	No	N/A	Autologous	2021	University Hospitals Birmingham NHS Foundation Trust, Birmingham Cambridge University Hospitals NHS Foundation Trust, Cambridge The Leeds Teaching Hospitals NHS Trust, Leeds University College London Hospital (UCLH), London Guy's and St Thomas' NHS Foundation Trust, London The Royal Marsden NHS Foundation Trust, London The Christie NHS Foundation Trust, Manchester Freeman Hospital, Newcastle Upon Tyne University Hospital Southampton NHS Foundation Trust, Southampton
Adaptimmune Therapeutics Plc	AFPC332T in Advanced HCC	NCT03132792	Phase I	In follow-up	Oncology (Other)	Hepatocellular Carcinoma Liver Cancer	T cells	Yes, *ex vivo*	Lentivirus	Autologous	2017	The Beatson West of Scotland Cancer Centre, Glasgow Guy's and St Thomas' NHS Foundation Trust, London UCLH Clinical Research Facility, London The Christie NHS Foundation Trust, Manchester
Adaptimmune Therapeutics Plc	Spearhead 1 Study in Subjects with Advanced Synovial Sarcoma or Myxoid/Round Cell Liposarcoma	GDC30020736	Phase II	In follow-up	Oncology (Other)	Synovial Sarcoma, Myxoid/round cell liposarcoma	T cells	Yes, *ex vivo*	Lentivirus	Autologous	2020	University College London Hospital (UCLH), London The Christie NHS Foundation Trust, Manchester
Adaptimmune Therapeutics Plc	ADP-A2M4CD8 in HLA-A2+ Subjects With MAGE-A4 Positive Esophageal or Esophagogastric Junction Cancers (SURPASS-2)	GDC30024054	Phase II	In follow-up	Oncology (Other)	Oesophageal Cancer	T cells	Yes, *ex vivo*	Lentivirus	Autologous	2021	The Christie NHS Foundation Trust, Manchester The Beatson West of Scotland Cancer Centre, Glasgow, Guy's and St Thomas NHS Foundation Trust, London University College London Hospital (UCLH), London

Cell and Gene Therapy Catapult ATMP Clinical Trials Database 2023

Sponsor	Title	Clinical Database Numbers	Trial Phase	Trial Status	Disease Area	Indication	Cell Type	Gene Modification / Gene Therapy	Type of Vector Used (if applicable)	Autologous/ Allogeneic	Year Trial Started	United Kingdom Site(s)
Adaptimmune Therapeutics Plc	ADP-A2M4CD8 Monotherapy and in Combination with Nivolumab in HLA-A2+ Subjects with MAGE-A4 Positive Ovarian Cancer (SURPASS-3)	GDC30029469	Phase II	Recruiting	Oncology (Other)	Head And Neck Cancer Squamous Cell Carcinoma Oral Cavity (Mouth) Cancer Oropharyngeal Cancer Hypopharyngeal Cancer Laryngeal Cancer Recurrent Head And Neck Cancer Squamous Cell Carcinoma	T cells	Yes, *ex vivo*	Lentivirus	Autologous	2023	University College London Hospital (UCLH), London The Royal Marsden NHS Foundation Trust, London The Christie NHS Foundation Trust, Manchester
Adverum Biotechnologies Inc	Safety and Efficacy of ADVM-022 in Treatment-Experienced Patients With Neovascular Age-related Macular Degeneration [LUNA]	GDC30024788	Phase II	Recruiting	Ophthalmology	Age-related Macular Degeneration (AMD)	N/A	Yes, *in vivo*	AAV	N/A	2022	Adverum Clinical Site 600, London Adverum Clinical Site 601, Oxford
Akamis Bio Ltd	A Study of NG-641 and Pembrolizumab in Squamous Cell Carcinoma of the Head and Neck (MOAT)	NCT04830592	Phase I	Recruiting	Oncology (Other)	Squamous Cell Carcinoma of the Head and Neck	N/A	Yes, *in vivo*	Oncolytic Virus	N/A	2021	Cardiff & Vale University Local Health Board, Cardiff Clatterbridge Cancer Centre NHS Foundation Trust, Liverpool The Royal Marsden NHS Foundation Trust, London University Hospital Southampton NHS Foundation Trust, Southampton
Akamis Bio Ltd	Study of NG-641 in Combination with Nivolumab in Metastatic or Advanced Epithelial Tumours (NEBULA)	NCT05043714	Phase I	Recruiting	Oncology (Other)	Metastatic Cancer Epithelial Tumor Transitional Cell Carcinoma (Urothelial Cell Carcinoma) Non-Small Cell Lung Cancer Endometrial Cancer Cervical Cancer Esophageal Cancer Gastric Cancer Triple-Negative Breast Cancer (TNBC) Hepatocellular Carcinoma Squamous Cell Carcinoma Uterine Cancer Recurrent Head And Neck Cancer Squamous Cell Carcinoma	N/A	Yes, *in vivo*	Adenovirus	N/A	2021	Oxford University Hospital, Oxford Clatterbridge Hospital, Liverpool
Akamis Bio Ltd	Study of NG-350A Plus Pembrolizumab in Metastatic or Advanced Epithelial Tumours (FORTIFY) (FORTIFY)	NCT05165433	Phase I	Recruiting	Oncology (Other)	Multiple Myeloma (Kahler Disease)	N/A	Yes, *in vivo*	Adenovirus	N/A	2022	Churchill Hospital, Oxford Clatterbridge Hospital, Liverpool
Aleta Biotherapeutics Inc	Phase I/IIA, First-In-Human Study of ALETA-001 In Subjects with B-Cell Lymphoma/Leukemia	GDC30028054	Phase I/II	In planning/ set-up	Oncology (Haematology)	B-Cell Non-Hodgkin Lymphoma	T cells	Yes, *ex vivo*	Lentivirus	Autologous	2022	The Christie NHS Foundation Trust, Manchester
AlloVir Inc	Phase 2 Multicenter, Randomized, Double-blind, Placebo-controlled Study to Assess the Safety and Efficacy of Viralym-M Compared to Placebo for the Prevention of AdV, BKV, CMV, EBV, HHV-6, and JCV Infection and/or Disease, in High-Risk Patients After Allogeneic Hematopoietic Cell Transplant	2021-003450-22 NCT04693637	Phase II	In follow-up	Infectious Disease	High-Risk Allogeneic Hematopoietic Cell Transplant patients	T cells	No	N/A	Allogeneic	2021	Unknown UK site(s)

Cell and Gene Therapy Catapult ATMP Clinical Trials Database 2023

Sponsor	Title	Clinical Database Numbers	Trial Phase	Trial Status	Disease Area	Indication	Cell Type	Gene Modification / Gene Therapy	Type of Vector Used (if applicable)	Autologous/ Allogeneic	Year Trial Started	United Kingdom Site(s)
AlloVir Inc	Study to Evaluate Viralym-M (ALVR105) for the Treatment of Virus-Associated Hemorrhagic Cystitis (HC)	GDC30021072	Phase III	Recruiting	Renal and Urogenital	Hemorrhagic Cystitis	T cells	No	N/A	Allogeneic	2021	Queen Elizabeth University Hospital, London Nottingham University Hospitals NHS Trust, Nottingham University Hospitals Bristol and Weston NHS Foundation Trust, Bristol Great Ormond Street Hospital for Children NHS Trust, London Hammersmith Hospital, London The Royal Marsden NHS Foundation Trust, London University College London Hospital (UCLH), London
AlloVir Inc	Posoleucel (ALVR105) for the Treatment of Adenovirus Infection in Pediatric and Adult Participants Receiving Standard of Care Following Allogeneic Hematopoietic Cell Transplantation	GDC30026126	Phase III	Recruiting	Infectious Disease	Adenoviridae Infections	T cells	No	N/A	Allogeneic	2022	Queen Elizabeth University Hospital, Glasgow Kings College Hospital NHS Foundation Trust, London Bristol Haematology and Oncology Centre, Bristol Royal Hospital for Children, Glasgow Birmingham Childrens Hospital NHS Foundation Trust, Birmingham University College London Hospital (UCLH), London St Mary's Hospital, London
AlloVir Inc	Study of Posoleucel (ALVR105, Viralym-M) for Multi-Virus Prevention in Patients Post-Allogeneic Hematopoietic Cell Transplant (Prevent)	GDC20033940	Phase II/III	Recruiting	Infectious Disease	Treatment for 6 viral pathogens potentially transmitted through transplantation	T cells	No	N/A	Allogeneic	2022	Bristol Haematology and Oncology Centre, Bristol Addenbrookes Hospital, Cambridge Queen Elizabeth University Hospital, Glasgow Great Ormond Street Hospital for Children NHS Trust, London
AlloVir Inc	Global Registry for Long-term Follow-up of Patients Participating in Clinical Trials with Posoleucel (ALVR105)	EudraCT-2022-000763-45	Phase IV	Recruiting	Infectious Disease	Treatment for 6 viral pathogens potentially transmitted through transplantation	T cells	No	N/A	Allogeneic	2022	Bristol Haematology and Oncology Centre, Bristol University College London Hospital (UCLH), London Birmingham Childrens Hospital NHS Foundation Trust, Birmingham Kings College Hospital NHS Foundation Trust, London Bristol Royal Hospital for Children, Bristol Sheffield Childrens Hospital, Sheffield Royal Manchester Childrens Hospital, Manchester Great Ormond Street Hospital for Children NHS Trust, London Nottingham University Hospitals NHS Trust, Nottingham Royal Marsden NHS Foundation Trust, London Queen Elizabeth Hospital, Birmingham St Marys Hospital, London Royal Hospital for Children, Glasgow
Amgen Inc	Postmarketing Prospective Study of Melanoma Patients Treated with Imlygic to Characterize Risk of Herpetic Infection	NCT02910557	Phase IV	Recruiting	Oncology (Other)	Melanoma	N/A	Yes, *in vivo*	Oncolytic Virus	N/A	2017	Cheltenham General Hospital, Cheltenham Royal Marsden NHS Foundation Trust, London Churchill Hospital, Oxford
Amgen Inc	Talimogene Laherparepvec with Pembrolizumab in Melanoma Following Progression on Prior Anti-PD-1 Based Therapy (MASTERKEY-115)	NCT04068181	Phase II	In follow-up	Oncology (Other)	Melanoma	N/A	Yes, *in vivo*	Oncolytic Virus	N/A	2020	Guy's and St Thomas NHS Foundation Trust, London The Royal Marsden NHS Foundation Trust, London
Amphera BV	Study on Dendritic Cell Therapy as Maintenance Therapy for Patients That Have Been Treated for Mesothelioma	EudraCT-2017-001774-41	Phase II/III	Completed	Oncology (Other)	Malignant Pleural Mesothelioma Malignant Mesothelioma	Antigen presenting cells	No	N/A	Autologous	2018	University of Leicester, Leicester

Cell and Gene Therapy Catapult ATMP Clinical Trials Database 2023

Sponsor	Title	Clinical Database Numbers	Trial Phase	Trial Status	Disease Area	Indication	Cell Type	Gene Modification / Gene Therapy	Type of Vector Used (if applicable)	Autologous/ Allogeneic	Year Trial Started	United Kingdom Site(s)
Astellas Gene Therapies	Gene Transfer Clinical Study in X-linked Myotubular Myopathy (ASPIRO)	GDC30011439	Phase I/II	Suspended	Neuromuscular	X-Linked Myotubular Myopathy (XLMTM or MTM)	N/A	Yes, *in vivo*	AAV	N/A	2017	Great Ormond Street Hospital for Children NHS Trust, London
Astellas Gene Therapies	Gene Transfer Study in Patients with Late Onset Pompe Disease (FORTIS)	NCT04174105	Phase I/II	Recruiting	Metabolic (Other)	Pompe Disease	N/A	Yes, *in vivo*	AAV	N/A	2020	Newcastle Upon Tyne Hospitals NHS Foundation Trust, Newcastle Upon Tyne
Astellas Pharma Inc	A Safety Surveillance Study in Subjects with Macular Degenerative Disease Treated with Human Embryonic Stem Cell-derived Retinal Pigment Epithelial Cell Therapy	GDC30013032	Phase I/II	Recruiting	Ophthalmology	Age-related Macular Degeneration (AMD)	Retinal	No	N/A	Allogeneic	2018	Site GB44001, London, United Kingdom
Atamyo Therapeutics SAS	GNT0006 Gene Therapy Trial in Patients with Limb-girdle Muscular Dystrophy	EudraCT-2021-004276-33	Phase I/II	Recruiting	Genetic Disorders	FKRP-related Limb-girdle Muscular Dystrophy (LGMDR9, Formerly LGMD2I)	N/A	Yes, *in vivo*	AAV	N/A	2022	Royal Victoria Infirmary, Newcastle Upon Tyne Newcastle Upon Tyne Hospitals NHS Foundation Trust, Newcastle Upon Tyne
Atara Biotherapeutics Inc	Tabelecleucel for Solid Organ or Allogeneic Hematopoietic Cell Transplant Participants With Epstein-Barr Virus-Associated Post-Transplant Lymphoproliferative Disease (EBV+ PTLD) After Failure of Rituximab or Rituximab and Chemotherapy (ALLELE)	GDC20006704	Phase III	Recruiting	Oncology (Other)	Epstein-Barr Virus-Associated Post-Transplant Lymphoproliferative Disease	T cells	No	N/A	Allogeneic	2020	Imperial College Healthcare NHS Trust, London Queen Elizabeth Hospital, Birmingham, Kings College Hospital NHS Foundation Trust, London
Atara Biotherapeutics Inc	A Study to Evaluate Tabelecleucel in Participants with Epstein-barr Virus-associated Diseases	GDC30021629	Phase II	Recruiting	Infectious Disease	Epstein-Barr Virus-Associated Diseases	T cells	No	N/A	Allogeneic	2021	Great Ormond Street Hospital for Children NHS Trust, London University Hospital Birmingham NHS Foundation Trust, Birmingham
Athersys Inc	MultiStem Administration for Stroke Treatment and Enhanced Recovery Study (MASTERS-2)	GDC20007216	Phase III	Recruiting	Cardiovascular	Acute Ischemic Stroke	Mesenchymal stem/stromal cells	No	N/A	Allogeneic	2021	Unknown UK site(s)

Cell and Gene Therapy Catapult ATMP Clinical Trials Database 2023

Sponsor	Title	Clinical Database Numbers	Trial Phase	Trial Status	Disease Area	Indication	Cell Type	Gene Modification / Gene Therapy	Type of Vector Used (if applicable)	Autologous/ Allogeneic	Year Trial Started	United Kingdom Site(s)
Autolus Therapeutics Plc	Phase I/II Study Evaluating AUTO4 in Patients with TRBC1 Positive T Cell Lymphoma	GDC30012898	Phase I/II	Recruiting	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)	T cells	Yes, *ex vivo*	Retrovirus	Autologous	2018	The Christie NHS Foundation Trust, Manchester Freeman Hospital, Newcastle Upon Tyne Manchester Royal Infirmary, Manchester University College London Hospital (UCLH), London The Beatson West of Scotland Cancer Centre, Glasgow Newcastle Upon Tyne Hospital, Newcastle
Autolus Therapeutics Plc	CD19/22 CAR T Cells (AUTO3) for the Treatment of Diffuse Large B Cell Lymphoma (ALEXANDER)	EudraCT-2016-004682-11	Phase I/II	In follow-up	Oncology (Haematology)	B-Cell lymphoma	T cells	Yes, *ex vivo*	Retrovirus	Autologous	2017	Freeman Hospital, Newcastle Upon Tyne The Christie NHS Foundation Trust, Manchester University College London Hospital (UCLH), London The Beatson West of Scotland Cancer Centre, Glasgow
Autolus Therapeutics Plc	Long-term Follow-up of Patients Treated with Autologous T Cells Genetically Modified	NCT03628612	Phase II	Recruiting	Oncology (Other)	Multiple Myeloma	T cells	Yes, *ex vivo*	Retrovirus	Autologous	2018	Great Ormond Street Hospital for Children NHS Foundation Trust, London University College London Hospital (UCLH), London Royal Manchester Children's Hospital, Manchester Freeman Hospital, Newcastle Upon Tyne
Autolus Therapeutics Plc	A Study of CD19 Targeted CAR T Cell Therapy in Adult Patients with Relapsed or Refractory B Cell Acute Lymphoblastic Leukaemia (ALL)	EudraCT-2019-001937-16	Phase I/II	In follow-up	Oncology (Haematology)	B-Cell Acute Lymphocytic Leukemia	T cells	Yes, *ex vivo*	Lentivirus	Autologous	2020	Bristol Haematology and Oncology Centre, Bristol Queen Elizabeth Hospital NHS Foundation Trust, London University College London Hospital (UCLH), London Kings College Hospital NHS Foundation Trust, London Manchester Royal Infirmary Hospital, Manchester Freeman Hospital, Newcastle Upon Tyne Beatson West of Scotland Cancer Center, Glasgow
Barts Health NHS Trust	Use of Bone Marrow Derived Stem Cell and G-CSF with Circulatory Assistance in the Treatment of DCM (DCM-Support)	NCT03572660	Phase II	Recruiting	Cardiovascular	Dilated Cardiomyopathy	Bone marrow mononuclear cells	No	N/A	Autologous	2018	St Bartholomew's Hospital, London
Bayer AG	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"	GDC30010370	Phase I/II	In follow-up	Haematological	Hemophilia A (Factor VIII Deficiency)	N/A	Yes, *in vivo*	AAV	N/A	2018	Manchester Royal Infirmary, Manchester
Belfast Health and Social Care Trust	Repair of Acute Respiratory Distress Syndrome by Stromal Cell Administration (REALIST)	GDC30011729	Phase I/II	Recruiting	Respiratory	Acute Respiratory Distress Syndrome (ARDS) due to COVID-19 or other causes of ARDS	Mesenchymal stem/stromal cells	No	N/A	Allogeneic	2019	Royal Hospitals, Belfast Guy's & St Thomas' NHS Foundation Trust, London Kings College Hospital NHS Foundation Trust, London Queen Elizabeth Hospital, Birmingham Birmingham Heartlands Hospital, Birmingham Edinburgh Royal Infirmary, Edinburgh University College London Hospital (UCLH), London Manchester Royal Infirmary, Manchester Sunderland Hospital, Sunderland Liverpool Royal Infirmary, Liverpool Southampton Hospital, Southampton Wythenshawe Hospital, Manchester Aintree University Hospital, Liverpool

Cell and Gene Therapy Catapult ATMP Clinical Trials Database 2023

Sponsor	Title	Clinical Database Numbers	Trial Phase	Trial Status	Disease Area	Indication	Cell Type	Gene Modification / Gene Therapy	Type of Vector Used (if applicable)	Autologous/ Allogeneic	Year Trial Started	United Kingdom Site(s)
Bellicum Pharmaceuticals Inc	Safety Study of Gene Modified Donor T-cells Following TCR Depleted Stem Cell Transplant	NCT02065869	Phase I/II	In follow-up	Oncology (Haematology)	Haematological Malignancies	T cells	Yes, *ex vivo*	Retrovirus	Allogeneic	2020	Great Ormond Street Hospital for Children NHS Foundation Trust, London Great North Children's Hospital, Newcastle Upon Tyne Royal Free London NHS Foundation Trust, London Newcastle Upon Tyne Hospital, Newcastle Upon Tyne
Biogen Inc	Long-term Safety and Efficacy Follow-up of BIIB111 for the Treatment of Choroideremia and BIIB112 for the Treatment of X-Linked Retinitis Pigmentosa (SOLSTICE)	EudraCT-2017-003104-42	Phase III	Recruiting	Ophthalmology	Choroideremia	N/A	Yes, *in vivo*	AAV	N/A	2018	Unconfirmed UK sites
BioMarin Pharmaceutical Inc	Gene Therapy Study in Severe Haemophilia A Patients (270-201)	EudraCT-2014-003880-38	Phase I/II	In follow-up	Haematological	Hemophilia A (Factor VIII Deficiency)	N/A	Yes, *in vivo*	AAV	N/A	2015	Hammersmith Hospital, London The Royal London Hospital, London Basingstoke and North Hampshire Hospital, Basingstoke Queen Elizabeth Hospital, Birmingham Guy's and St Thomas' NHS Foundation Trust, London Glasgow Royal Infirmary, Glasgow Addenbrooke's Hospital, Cambridge Bristol Haematology and Oncology Centre, UHBW Bristol University Hospital Southampton NHS Foundation Trust, Southampton
BioMarin Pharmaceutical Inc	Single-arm Study To Evaluate The Efficacy and Safety of Valoctocogene Roxaparvovec in Hemophilia A Patients at A Dose of 4E13 vg/kg (GENEr8-2)	GDC30015191	Phase III	Completed	Haematological	Hemophilia A (Factor VIII Deficiency)	N/A	Yes, *in vivo*	AAV	N/A	2018	Addenbrooke's Hospital, Cambridge Glasgow Royal Infirmary, Glasgow Barts and The London School of Medicine and Dentistry, London Hammersmith Hospital, London Guy's and St Thomas' NHS Foundation Trust, London Churchill Hospital, Oxford University Hospital Southampton NHS Foundation Trust, Southampton Queen Elizabeth Hospital NHS Foundation Trustl, London
BioMarin Pharmaceutical Inc	Gene Therapy Study in Severe Haemophilia A Patients with Antibodies Against AAV5(GENEr8-AAV5+)	GDC30015673	Phase I/II	In follow-up	Haematological	Hemophilia A (Factor VIII Deficiency)	N/A	Yes, *in vivo*	AAV	N/A	2018	Royal Free London NHS Foundation Trust, London University Hospital Southampton NHS Foundation Trust, Southampton
BioMarin Pharmaceutical Inc	AAV Gene Therapy Study for Subjects With PKU	GDC30021766	Phase I/II	Suspended	Metabolic (Other)	Phenylketonuria (PKU)	N/A	Yes, *in vivo*	AAV	N/A	2020	University Hospital Birmingham NHS Foundation Trust, Birmingham
BioMarin Pharmaceutical Inc	A Phase I/II Study to Evaluate the Safety and Efficacy of BMN 270 Gene Transfer in Patients with Severe Hemophilia a and Active or Prior Inhibitors	EudraCT-2019-003213-34	Phase I/II	Recruiting	Haematological	Hemophilia A (Factor VIII Deficiency)	N/A	Yes, *in vivo*	AAV	N/A	2020	Royal Free London NHS Foundation Trust, London Queen Elizabeth Hospital, Birmingham Guy's and St Thomas' NHS Foundation Trust, London

Cell and Gene Therapy Catapult ATMP Clinical Trials Database 2023

Sponsor	Title	Clinical Database Numbers	Trial Phase	Trial Status	Disease Area	Indication	Cell Type	Gene Modification / Gene Therapy	Type of Vector Used (if applicable)	Autologous/ Allogeneic	Year Trial Started	United Kingdom Site(s)
BioMarin Pharmaceutical Inc	A Long-term Follow-up Study in Severe Hemophilia a Subjects Who Received Bmn 270 in a Prior Biomarin Clinical Trial (270-401)	NCT05768386	Phase IV	Recruiting	Haematological	Heamophilia A	N/A	Yes, *in vivo*	AAV	N/A	2023	St Bartholomew's Hospital, London
bluebird bio Inc	Long-term Follow-up of Participants with Cerebral Adrenoleukodystrophy who were treated with Lenti-D Drug Product	EudraCT-2015-002805-13	Phase III	In follow-up	Metabolic (Other)	Adrenoleukodystrophy	CD34 and/or CD133 stem cells	Yes, *ex vivo*	Lentivirus	Autologous	2016	Great Ormond Street Hospital for Children NHS Foundation Trust, London
bluebird bio Inc	A Study Evaluating Gene Therapy with BB305 Lentiviral Vector in Sickle Cell Disease	GDC30019015	Phase III	Recruiting	Haematological	Sickle Cell Disease	CD34 and/or CD133 stem cells	Yes, *ex vivo*	Lentivirus	Autologous	2020	Unknown UK site(s)
bluebird bio Inc	A Clinical Study to Assess the Efficacy and Safety of Gene Therapy for the Treatment of Cerebral Adrenoleukodystrophy (CALD)	GDC50004392	Phase III	In follow-up	Metabolic (Other)	Adrenoleukodystrophy	CD34 and/or CD133 stem cells	Yes, *ex vivo*	Lentivirus	Autologous	2019	Central Manchester University Hospitals NHS Foundation Trust, Manchester University College London Hospital (UCLH), London
bluebird bio Inc	Long-term Follow-up of Subjects With Transfusion-Dependent ß-Thalassemia (TDT) Treated With Ex Vivo Gene Therapy	NCT02633943	Phase III	In follow-up	Haematological	Sickle Cell Disease β-Thalassemia	CD34 and/or CD133 stem cells	Ye, *ex vivo*	Lentivirus	Autologous	2013	University College London Hospital (UCLH), London
Boston Children's Hospital	Gene Transfer for Severe Combined Immunodeficiency, X-linked (SCID-X1) Using a Self-inactivating (SIN) Gammaretroviral Vector	NCT01129544	Phase I/II	Completed	Inflammatory and immune system	X-Linked SCID Severe Combined Immune Deficiency (SCID)	CD34 and/or CD133 stem cells	Yes, *ex vivo*	Retrovirus	Autologous	2010	Great Ormond Street Hospital for Children NHS Foundation Trust, London
Boston Children's Hospital	Phase I/II Trial of Lentiviral Gene Transfer for SCID-X1 with Low Dose Targeted Busulfan Conditioning	NCT03311503	Phase I/II	In follow-up	Inflammatory and immune system	X-Linked SCID Severe Combined Immune Deficiency (SCID)	CD34 and/or CD133 stem cells	Yes, *ex vivo*	Lentivirus	Autologous	2018	Great Ormond Street Hospital for Children NHS Foundation Trust, London

Cell and Gene Therapy Catapult ATMP Clinical Trials Database 2023

Sponsor	Title	Clinical Database Numbers	Trial Phase	Trial Status	Disease Area	Indication	Cell Type	Gene Modification / Gene Therapy	Type of Vector Used (if applicable)	Autologous/ Allogeneic	Year Trial Started	United Kingdom Site(s)
Bristol-Myers Squibb	A Study to Evaluate the Safety and Tolerability of BMS-986403 in Participants With Relapsed and/or Refractory Chronic Lymphocytic Leukemia or Small Lymphocytic Lymphoma	CA097-001	Phase I	Terminated	Oncology (Haematology)	Relapsed/Refractory Follicular Lymphoma	T cells	Yes, *ex vivo*	Lentivirus	Autologous	2022	Cambridge University Hospitals NHS Foundation Trust, Cambridge
Cambridge University Hospitals NHS Foundation Trust & University of Cambridge Lund University Region Skåne - Skåne University Hospital	STEM-PD trial: A multicentre, single arm, first in human, dose-escalation trial, investigating the safety and tolerability of intraputamenal transplantation of human embryonic stem cell derived dopaminergic cells for Parkinson's disease (STEM-PD product)	IRAS: 297753 EudraCT: 2021-001366-38	Phase I/II	Recruiting	Neurological	Parkinson's Disease	Other	No	N/A	Allogeneic	2022	Cambridge University Hospitals NHS Foundation Trust, Cambridge
Cancer Research UK	A Cancer Research UK Phase I/IIa trial of Chimpanzee Adenovirus Oxford 1 (CHADOX1) and Modified Vaccinia Ankara (MVA) vaccines against MAGE-A3 and NY-ESO-1 with standard of care treatment (chemotherapy and an immune checkpoint inhibitor)	NCT04908111	Phase I/II	Recruiting	Oncology (Other)	Non-Small Cell Lung Cancer and Gastro-Oesophageal Cancer	N/A	Yes, *in vivo*	Adenovirus	N/A	2021	The Christie NHS Foundation Trust, Manchester St James' University Hospital, Leeds Southampton General Hospital, Southampton The Clatterbridge Cancer Centre NHS Foundation Trust, Liverpool Churchill Hospital, Oxford Guy's and St Thomas' NHS Foundation Trust, London Queen Elizabeth Hospital, Birmingham Leicester Royal Infirmary, Leicester
Cancer Vaccines Ltd	Investigation of a Therapeutic Vaccine (ACIT-1) in Cancer	GDC50001331	Phase I/II	In follow-up	Oncology (Other)	Pancreatic cancer	Antigen presenting cells	No	N/A	Allogeneic	2017	Royal Liverpool University Hospital, Liverpool
Celgene Corp	A Study to Compare the Efficacy and Safety of JCAR017 to Standard of Care in Adult Subjects with High-risk, Transplant-eligible Relapsed or Refractory Aggressive B-cell Non-Hodgkin Lymphomas (TRANSFORM)	GDC30008058	Phase III	Completed	Oncology (Haematology)	B-Cell Non-Hodgkin Lymphoma	T cells	Yes, *ex vivo*	Lentivirus	Autologous	2018	The Clatterbridge Cancer Centre NHS Foundation Trust, Liverpool
Celgene Corp	Efficacy and Safety Study of Bb2121 Versus Standard Regimens in Subjects with Relapsed and Refractory Multiple Myeloma (RRMM) (KarMMa-3)	GDC30016225	Phase III	In follow-up	Oncology (Haematology)	Multiple Myeloma	T cells	Yes, *ex vivo*	Lentivirus	Autologous	2019	University College London (UCL) Cancer Institute, London University Hospital Southampton NHS Foundation Trust, Southampton
Celgene Corp	Long-Term Follow-up Protocol for Subjects Treated With Gene-Modified T Cells	NCT03435796	Phase II/III	Recruiting	Oncology (Other)	Neoplasms	T cells	Yes, *ex vivo*	Lentivirus	Autologous	2019	The Leeds Teaching Hospitals NHS Trust, Leeds

Cell and Gene Therapy Catapult ATMP Clinical Trials Database 2023

Sponsor	Title	Clinical Database Numbers	Trial Phase	Trial Status	Disease Area	Indication	Cell Type	Gene Modification / Gene Therapy	Type of Vector Used (if applicable)	Autologous/ Allogeneic	Year Trial Started	United Kingdom Site(s)
Celgene Corp	Trial to Determine the Efficacy and Safety of JCAR017 in Adult Participants with Aggressive B-cell Non-hodgkin Lymphoma (TRANSCENDWORLD)	NCT03484702	Phase II	In follow-up	Oncology (Haematology)	B-Cell Non-Hodgkin Lymphoma	T cells	Yes, *ex vivo*	Lentivirus	Autologous	2018	University College London (UCL) Cancer Institute, London The Christie NHS Foundation Trust, Manchester
Celgene Corp	An Efficacy and Safety Study of bb2121 in Subjects with Relapsed and Refractory Multiple Myeloma and in Subjects with High-Risk Multiple Myeloma (KarMMa-2)	NCT03601078	Phase II	In follow-up	Oncology (Haematology)	Multiple Myeloma	T cells	Yes, *ex vivo*	Lentivirus	Autologous	2019	King's College Hospital, London
Celgene Corp	A Study to Evaluate the Efficacy and Safety of Jcar017 in Adult Subjects With Relapsed or Refractory Indolent B-cell Non-hodgkin Lymphoma (NHL) (Transcend FL)	NCT04245839	Phase II	Recruiting	Oncology (Haematology)	Follicular Lymphoma B-Cell Non-Hodgkin Lymphoma Marginal Zone B-cell Lymphoma Nodal Marginal Zone B-Cell Lymphoma	T cells	Yes, *ex vivo*	Lentivirus	Autologous	2020	The Christie NHS Foundation Trust, Manchester University College London Hospital (UCLH), London
CellProthera SAS	EXCELLENT (EXpanded CELL ENdocardiac Transplantation) (EXCELLENT)	GDC40003010	Phase I/II	In follow-up	Cardiovascular	Myocardial Infarction Congestive Heart Failure (Heart Failure)	CD34 and/or CD133 stem cells	No	N/A	Autologous	2015	The Leeds Teaching Hospitals NHS Trust, Leeds St Bartholomews Hospital, London Ninewells Hospital and Medical School, Dundee University of Edinburgh, Edinburgh Queen Elizabeth Hospital NHS Foundation Trust, London
Celyad Oncology SA	alloSHRINK - Standard Chemotherapy Regimen and Immunotherapy with Allogeneic NKG2D-based CYAD-101 Chimeric Antigen Receptor T-cells (alloSHRINK)	NCT03692429	Phase I	Terminated	Oncology (Other)	Metastatic Colorectal Cancer	T cells	Yes, *ex vivo*	Retrovirus	Allogeneic	2018	Guy's and St Thomas' NHS Foundation Trust, London The Christie NHS Foundation Trust, Manchester
Cook MyoSite Inc	Adaptive Design Study of Autologous Muscle Derived Cells Compared to Placebo for Female Urinary Sphincter Repair (CELLEBRATE)	NCT03104517	Phase III	Recruiting	Renal and Urogenital	Stress Urinary Incontinence	Skeletal Muscle	No	N/A	Autologous	2018	Hull Royal Infirmary, Hull St. James's University Hospital, Leeds Derriford Hospital, Plymouth
CSL Ltd	HOPE-B: Trial of AMT-061 in Severe or Moderately Severe Hemophilia B Patients	GDC30015761	Phase III	In follow-up	Haematological	Haemophilia B	N/A	Yes, *in vivo*	AAV	N/A	2018	University Hospitals Bristol and Weston NHS Foundation Trust, Bristol Addenbrooke's Hospital, Cambridge The Royal London Hospital, London University Hospital Southampton NHS Foundation Trust, Southampton

Cell and Gene Therapy Catapult ATMP Clinical Trials Database 2023

Sponsor	Title	Clinical Database Numbers	Trial Phase	Trial Status	Disease Area	Indication	Cell Type	Gene Modification / Gene Therapy	Type of Vector Used (if applicable)	Autologous/ Allogeneic	Year Trial Started	United Kingdom Site(s)
Decibel Therapeutics Inc	A Study of DB-OTO, an AAV Based Gene Therapy, in Children/Infants With Hearing Loss Due to Otoferlin Mutations (CHORD)	GDC30028730	Phase I/II	Recruiting	Ear, nose, and throat	Hearing Loss Due to Otoferlin Mutations (CHORD)	N/A	Yes, *in vivo*	AAV	N/A	2023	Cambridge University Hospitals NHS Foundation Trust, Cambridge Great Ormond Street Hospital for Children NHS Foundation Trust, London
Freeline Therapeutics Holdings Plc	A Long-Term Follow-Up Study of Haemophilia B Patients Who Have Undergone Gene Therapy	NCT03641703	Phase I/II	Terminated	Haematological	Haemophilia B	N/A	Yes, *in vivo*	AAV	N/A	2018	Royal Free London NHS Foundation Trust, London Oxford University Hospitals NHS Foundation Trust, Oxford
Freeline Therapeutics Holdings Plc	A Long Term Follow-up Study of Fabry Disease Subjects Treated with FLT190	NCT04455230	Phase I/II	In follow-up	Metabolic (Other)	Fabry Disease Lysosomal Storage Disorders	N/A	Yes, *in vivo*	AAV	N/A	2020	Royal Free London NHS Foundation Trust, London
Freeline Therapeutics Holdings Plc	Phase I/II Dose Confirmation Study of FLT180a in Hemophilia B	GDC30026884	Phase I/II	Terminated	Genetic Disorders	Hemophilia B	N/A	Yes, *in vivo*	AAV	N/A	2021	Glasgow Royal Infirmary, Glasgow Guy's and St Thomas' NHS Foundation Trust, London Royal Free London NHS Foundation Trust, London Royal Victoria Infirmary, Newcastle
Freeline Therapeutics Holdings Plc	A Gene Therapy Study in Patients with Gaucher Disease Type 1 (GALILEO-1)	GDC30029468	Phase I/II	Recruiting	Genetic Disorders	Gaucher Disease Type 1	N/A	Yes, *in vivo*	AAV	N/A	2022	Queen Elizabeth Hospital, Birmingham Royal Free London NHS Foundation Trust, London Salford Royal Hospital, Salford
Gamida Cell Ltd	Stem Cell Transplantation With NiCord (Omidubicel) vs Standard Umbilical Cord Blood in Patients With Leukemia, Lymphoma, and Myelodysplastic Syndrome (MDS)	GDC30007817	Phase III	In follow-up	Oncology (Haematology)	Leukemia, Lymphoma, and Myelodysplastic Syndrome (MDS)	CD34 and/or CD133 stem cells	No	N/A	Allogeneic	2018	Royal Marsden NHS Foundation Trust, London Queen Elizabeth Hospital, Birmingham St Jame's Hospital, Leeds Manchester Royal Infirmary, Manchester Royal Manchester Children's Hospital, Manchester
Genethon SA	Gene Therapy for X-linked Chronic Granulomatous Disease (X-CGD) (CGD)	NCT01855685	Phase I/II	In follow-up	Inflammatory and immune system	Chronic Granulomatous Disease	CD34 and/or CD133 stem cells	Yes, *ex vivo*	Lentivirus	Autologous	2013	Great Ormond Street Hospital for Children NHS Foundation Trust, London Royal Free London NHS Foundation Trust, London University College London Hospital (UCLH), London

Cell and Gene Therapy Catapult ATMP Clinical Trials Database 2023

Sponsor	Title	Clinical Database Numbers	Trial Phase	Trial Status	Disease Area	Indication	Cell Type	Gene Modification / Gene Therapy	Type of Vector Used (if applicable)	Autologous/ Allogeneic	Year Trial Started	United Kingdom Site(s)
Genethon SA	Long Term Safety Follow up of Haematopoietic Stem Cell Gene Therapy for the Wiskott Aldrich Syndrome (WASFUP)	EudraCT-2014-000274-20	Phase I/II	In follow-up	Inflammatory and immune system	Wiskott-Aldrich Syndrome	CD34 and/or CD133 stem cells	Yes, *ex vivo*	Lentivirus	Autologous	2014	University College London (UCL) Institute of Child Health, London
Genethon SA	Phase I Single-arm, Dose-escalation Study of Microdystrophin Gene Transfer in Subjects with Duchenne Muscular Dystrophy (DMD)	GDC30017135	Phase I	Recruiting	Neuromuscular	Duchenne Muscular Dystrophy	N/A	Yes, *in vivo*	AAV	N/A	2021	Unknown UK site(s)
GenSight Biologics SA	Dose-escalation Study to Evaluate the Safety and Tolerability of GS030 in Subjects with Retinitis Pigmentosa (PIONEER)	GDC30011617	Phase I/II	Recruiting	Ophthalmology	Retinitis Pigmentosa (Retinitis)	N/A	Yes, *in vivo*	AAV	N/A	2017	Moorfields Eye Hospital NHS Foundation Trust, London
GenSight Biologics SA	Efficacy & Safety Study of Bilateral IVT Injection of GS010 in LHON Subjects Due to the ND4 Mutation for up to 1 Year (REFLECT)	NCT03293524	Phase III	In follow-up	Ophthalmology	Leber Hereditary Optic Neuropathy	N/A	Yes, *in vivo*	AAV	N/A	2019	Moorfields Eye Hospital NHS Foundation Trust, London
Gilead Sciences Inc	A Study to Compare Axicabtagene Ciloleucel With Standard of Care Therapy as First-line Treatment in Participants With High-risk Large B-cell Lymphoma(ZUMA-23)	NCT05605899	Phase III	Recruiting	Oncology (Other)	Follicular Lymphoma, Diffuse Large B-Cell Lymphoma, Marginal Zone B-cell Lymphoma	T cells	Yes, *ex vivo*	Retrovirus	Autologous	2023	Cambridge University Hospitals NHS Foundation Trust, Cambridge
Gilead Sciences Inc	Long-term Follow-up Study for Participants of Kite-sponsored Interventional Studies Treated with Gene-modified Cells	NCT05041309	Phase II	Recruiting	Oncology (Haematology)	Refractory Large B-Cell Lymphoma	T cells	Yes, *ex vivo*	Retrovirus	Autologous	2021	Unconfirmed UK site(s)
Gilead Sciences Inc	Long-term, Non-interventional Study of Recipients of Tecartus for Treatment of Adult Patients with Relapsed or Refractory Mantle Cell Lymphoma (MCL)	EUPAS45813	Phase IV	In planning/ set-up	Oncology (Haematology)	Mantle Cell Lymphoma	T cells	Yes, *ex vivo*	Retrovirus	Autologous	2022	Unconfirmed UK Site(s)

Cell and Gene Therapy Catapult ATMP Clinical Trials Database 2023

Sponsor	Title	Clinical Database Numbers	Trial Phase	Trial Status	Disease Area	Indication	Cell Type	Gene Modification / Gene Therapy	Type of Vector Used (if applicable)	Autologous/ Allogeneic	Year Trial Started	United Kingdom Site(s)
Great Ormond Street Hospital for Children NHS Trust	Lentiviral Gene Therapy for X-linked Severe Combined Immunodeficiency	NCT03601286	Phase I/II	Recruiting	Inflammatory and immune system	X-Linked SCID Severe Combined Immune Deficiency (SCID)	CD34 and/or CD133 stem cells	Yes, *ex vivo*	Lentivirus	Autologous	2018	Great Ormond Street Hospital for Children NHS Foundation Trust, London
Great Ormond Street Hospital for Children NHS Trust	TT52CAR19 Therapy for B-Cell Acute Lymphoblastic Leukaemia (B-ALL) (PBLTT52CAR19)	EudraCT-2019-003462-40	Phase I	In follow-up	Oncology (Haematology)	B-Cell Acute Lymphocytic Leukemia (B-Cell Acute Lymphoblastic Leukaemia)	T cells	Yes, *ex vivo*	Lentivirus	Allogeneic	2020	Great Ormond Street Hospital for Children NHS Foundation Trust, London
Great Ormond Street Hospital for Children NHS Trust	Base Edited CAR7 T Cells to Treat T Cell Malignancies (TvT CAR7) (TvT CAR7)	ISRCTN15323014	Phase I	Recruiting	Oncology (Haematology)	T-cell leukaemia	T cells	Yes, *ex vivo*	Non-viral	Allogeneic	2022	Great Ormond Street Hospital for Children NHS Foundation Trust, London
GSK plc	Master Protocol to Assess the Safety and Antitumor Activity of Genetiy Engineered T Cells in NY-ESO-1 And/Or Lage-1a Positive Solid Tumors (IGNYTE-ESO)	GDC20006945	Phase II	In follow-up	Oncology (Other)	Synovial Sarcoma Solid Tumor	T cells	Ye, *ex vivo*	Lentivirus	Autologous	2019	GSK Investigative Site, London The Christie NHS Foundation Trust, Manchester
GSK plc	Adoptive Cell Therapy Long-term Follow-up (LTFU) Study	NCT03391778	Phase I	Recruiting	Oncology (Other)	Various	T cells	Yes, *ex vivo*	Lentivirus	Autologous	2018	GSK Investigative Site, London GSK Investigative Site, Manchester GSK Investigative Site, Sutton
GSK plc	Pilot Immunotherapy Study with Letetresgene Autoleucel (Lete-cel, GSK3377794)T-cells in New York Esophageal Squamous Cell Carcinoma-1 (NY-ESO-1)/ LAGE-1a-positive Advanced Non-small Cell Lung Cancer (NSCLC) Either Alone or in Combination with Pembrolizumab	NCT03709706	Phase I/II	Terminated	Oncology (Other)	Non-Small Cell Lung Cancer	T cells	Yes, *ex vivo*	Lentivirus	Autologous	2019	GSK Investigative Site, London The Christie NHS Foundation Trust, Manchester
Gyroscope Therapeutics Holdings Ltd	Focus: A Phase I/II First in Human Study to Evaluate the Safety and Efficacy of GT005 Administered in Subjects with Dry AMD	NCT03846193	Phase I/II	Terminated	Ophthalmology	Age-related Macular Degeneration (AMD)	N/A	Yes, *in vivo*	AAV	N/A	2018	Southampton General Hospital, Southampton London Vision Clinic, London Moorfields Eye Hospital, London Sunderland Eye Infirmary, Sunderland Manchester Eye Hospital, Manchester Bristol Eye Hospital, UHBW Bristol Oxford University Hospital, Oxford

Cell and Gene Therapy Catapult ATMP Clinical Trials Database 2023

Sponsor	Title	Clinical Database Numbers	Trial Phase	Trial Status	Disease Area	Indication	Cell Type	Gene Modification / Gene Therapy	Type of Vector Used (if applicable)	Autologous/ Allogeneic	Year Trial Started	United Kingdom Site(s)
Gyroscope Therapeutics Holdings Ltd	EXPLORE: A Phase II Study to Evaluate the Safety and Efficacy of Two Doses of GT005 (EXPLORE)	EudraCT-2019-003421-22	Phase II	Terminated	Ophthalmology	Age-related Macular Degeneration (AMD)	N/A	Yes, *in vivo*	AAV	N/A	2020	Sunderland Eye Infirmary, Sunderland Bristol Eye Hospital, Bristol
Gyroscope Therapeutics Holdings Ltd	HORIZON : A Phase II Study to Evaluate the Safety and Efficacy of Two Doses of GT005	GDC30024812	Phase II	In follow-up	Ophthalmology	Age-related Macular Degeneration (AMD)	N/A	Yes, *in vivo*	AAV	N/A	2020	Sunderland Eye Infirmary, Sunderland Moorfields Eye Hospital, London John Radcliff Hospital, Oxford London Vision Clinic, London
Holostem Terapie Avanzate SRL	Follow-up Study After ACLSCT for Restoration of Corneal Epithelium in Patients with LSCD Due to Ocular Burns (HOLOCORE-FU)	NCT03288844	Phase IV	Completed	Ophthalmology	Burns Unspecified Ophthalmological Disorders	Corneal	No	N/A	Autologous	2017	Newcastle Upon Tyne Hospitals NHS Foundation Trust, Newcastle Upon Tyne Moorfields Eye Hospital, London University of Nottingham, Nottingham Queen's Medical Centre, Nottingham
Holostem Terapie Avanzate SRL	implant for restoration of corneal epithelium in patients with limbal stem cell deficiency due to ocular burns: observational study of routine clinical practice	CCD-GPLSCD01-05	Phase IV	Recruiting	Ophthalmology	Burns Unspecified Ophthalmological Disorders	Corneal	No	N/A	Autologous	2016	Newcastle Upon Tyne Hospitals NHS Foundation Trust, Newcastle Upon Tyne Moorfields Eye Hospital, London
Hospital of the University of Munich	Treatment of Viral Infections after Stem Cell Transplantation with Specific Immune Cells	EudraCT-2018-000853-29	Phase III	Recruiting	Infectious Disease	Cytomegalovirus (HHV-5) Infections Epstein–Barr Virus (HHV-4) Infections Adenoviridae Infections	T cells	No	N/A	Allogeneic	2019	Great North Children's Hospital, Newcastle Upon Tyne
Innovacell Biotechnologie AG	Skeletal Muscle-derived Cell Implantation for Treatment of Fecal Incontinence (Fidelia)	NCT04976153	Phase III	Recruiting	Gastrointestinal (Other)	Fecal Incontinence	Unknown	No	N/A	Autologous	2022	St Marys Hospital, London
Instil Bio Inc	OVSTAR TIL Trial (OVarian Cancer Co-STimulatory Antigen Receptor TIL Trial) (OVSTAR)	GDC50006939	Phase I/II	Withdrawn	Oncology (Other)	Metastatic Ovarian Cancer	T cells	Yes, *ex vivo*	Unknown	Autologous	2020	Queens Elizabeth Hospital, Birmingham The Christie NHS Foundation Trust, Manchester

Cell and Gene Therapy Catapult ATMP Clinical Trials Database 2023

Sponsor	Title	Clinical Database Numbers	Trial Phase	Trial Status	Disease Area	Indication	Cell Type	Gene Modification / Gene Therapy	Type of Vector Used (if applicable)	Autologous/ Allogeneic	Year Trial Started	United Kingdom Site(s)
Instil Bio Inc	ITIL-168 in Advanced Melanoma (DELTA-1)	GDC50009494	Phase II	Terminated	Oncology (Other)	Advanced Melanoma	T cells	No	N/A	Autologous	2021	Cambridge University Hospitals NHS Foundation Trust, Cambridge
Intellia Therapeutics Inc	Study to Evaluate Safety, Tolerability, Pharmacokinetics, and Pharmacodynamics of NTLA-2001 in Patients with Hereditary Transthyretin Amyloidosis with Polyneuropathy (ATTRV-PN)	GDC30025382	Phase I	Recruiting	Metabolic (Other)	Hereditary Transthyretin Amyloidosis With Polyneuropathy (ATTRv-PN)	N/A	Yes, *in vivo*	Non-viral	N/A	2020	Clinical Trial Site, London
Intellia Therapeutics Inc	NTLA-2002 in Adults with Hereditary Angioedema (HAE) (NTLA-2002)	GDC30028978	Phase I/II	Recruiting	Genetic Disorders	Hereditary Angioedema (HAE) (C1 Esterase Inhibitor [C1-INH] Deficiency)	N/A	Yes, *in vivo*	Non-viral	N/A	2021	Cambridge University Hospitals NHS Foundation Trust, Cambridge Addenbrookes Hospital, Cambridge
Iovance Biotherapeutics Inc	Study of LN-145, Autologous Tumor Infiltrating Lymphocytes in the Treatment of Patients With Cervical Carcinoma	GDC30007582	Phase II	Recruiting	Oncology (Other)	Cervical Carcinomas	T cells	No	N/A	Autologous	2017	Bristol Haematology and Oncology Centre, Bristol Sarah Cannon Research Institute, London University College London Hospital (UCLH), London NHS Greater Glasgow and Clyde, Glasgow
Iovance Biotherapeutics Inc	Study of Autologous Tumor Infiltrating Lymphocytes in Patients with Solid Tumors	NCT03645928	Phase II	Recruiting	Oncology (Other)	Metastatic Melanoma	T cells	No	N/A	Autologous	2019	Guy's and St Thomas' NHS Foundation Trust, London Bristol Haematology and Oncology Centre, Bristol The Royal Marsden NHS Foundation Trust, London
Iovance Biotherapeutics Inc	Autologous LN-145 in Patients With Metastatic Non-Small-Cell Lung Cancer	GDC30024884	Phase II	Recruiting	Oncology (Other)	Non Small Cell Lung Cancer	T cells	No	N/A	Autologous	2022	Bristol Haematology and Oncology Centre, Bristol
Ixaka Ltd	The Efficacy and Safety of REX-001 to Treat Ischemic Ulcers in Subjects with CLI Rutherford Category 5 and DM	NCT03174522	Phase III	Terminated	Cardiovascular	Critical Limb Ischemia in patients with Diabetes Mellitus	Bone marrow mononuclear cells	No	N/A	Autologous	2017	Addenbrooke's Hospital, Cambridge University Hospitals Birmingham NHS Foundation Trust, Birmingham University Hospital of Wales, Cardiff Freeman Hospital, Newcastle Upon Tyne

Cell and Gene Therapy Catapult ATMP Clinical Trials Database 2023

Sponsor	Title	Clinical Database Numbers	Trial Phase	Trial Status	Disease Area	Indication	Cell Type	Gene Modification / Gene Therapy	Type of Vector Used (if applicable)	Autologous/ Allogeneic	Year Trial Started	United Kingdom Site(s)
Johnson & Johnson	A Study Comparing JNJ-68284528, a CAR-T Therapy Directed Against B-cell Maturation Antigen (BCMA), Versus Pomalidomide, Bortezomib and Dexamethasone (PVd) or Daratumumab, Pomalidomide and Dexamethasone (DPd) in Participants with Relapsed and Lenalidomide-Refractory Multiple Myeloma (CARTITUDE-4)	NCT04181827	Phase III	In follow-up	Oncology (Haematology)	Multiple Myeloma	T cells	Yes,, *ex vivo*	Lentivirus	Autologous	2020	Queen Elizabeth Hospital, Birmingham Bristol Haematology and Oncology Centre, Bristol University College London Hospital (UCLH), London King's College Hospital, London The Christie NHS Foundation Trust, Manchester Freeman Hospital, Newcastle Upon Tyne Cardiff and Vale University Hospital, Cardiff
Johnson & Johnson	A Study of Bortezomib, Lenalidomide and Dexamethasone (VRd) Followed by Cilta-cel, a CAR-T Therapy Directed Against BCMA Versus VRd Followed by Lenalidomide and Dexamethasone (Rd) Therapy in Participants With Newly Diagnosed Multiple Myeloma for Whom ASCT is Not Planned as Initial Therapy	NCT04923893	Phase III	Recruiting	Oncology (Haematology)	Multiple Myeloma	T cells	Yes, *ex vivo*	Lentivirus	Autologous	2022	University Hospitals Birmingham NHS Trust, Birmingham Bristol Royal Infirmary, Bristol Leeds Teaching Hospitals NHS Trust, Leed University College London Hospital (UCLH), London King's College Hospital, London Manchester Royal Infirmary, Manchester The Royal Marsden NHS Trust Sutton, Surrey
Johnson & Johnson	A Study to Evaluate Intravitreal JNJ-81201887 (AAVCAGsCD59) Compared to Sham Procedure for the Treatment of Geographic Atrophy (GA) Secondary to Age-related Macular Degeneration (AMD)	NCT05811351	Phase II	Recruiting	Ophthalmology	Dry (Atrophic) Macular Degeneration	N/A	Yes, *in vivo*	AAV	N/A	2023	The Retina Clinic, London Oxford Eye Hospital, Oxford
King's College London	Phase I Trial: T4 Immunotherapy of Head and Neck Cancer	NCT01818323	Phase I	Recruiting	Oncology (Other)	Head And Neck Cancer	T cells	Yes, *ex vivo*	Retrovirus	Autologous	2015	Guy's and St Thomas' NHS Foundation Trust, London
King's College London Guy's and St Thomas' NHS Foundation Trust	Treg Immunotherapy in Crohn's Disease (TRIBUTE)	ISRCTN97547683	Phase I/II	Recruiting	Gastrointestinal (Other)	Crohn's Disease	T cells	No	N/A	Autologous	2022	Guy's and St Thomas' NHS Foundation Trust, London
Kite Pharma Inc	Study of Effectiveness of Axicabtagene Ciloleucel Compared to Standard of Care Therapy in Patients with Relapsed/ Refractory Diffuse Large B Cell Lymphoma (ZUMA-7)	GDC40002568	Phase III	In follow-up	Oncology (Haematology)	B-Cell Lymphoma	T cells	Yes, ex vivo	Retrovirus	Autologous	2018	University College London Hospital (UCLH), London The Christie NHS Foundation Trust, Manchester Barts Health NHS Trust, London University Hospitals Birmingham NHS Foundation Trust, Birmingham The Royal Marsden NHS Foundation Trust, Sutton
Kite Pharma Inc	A Phase 3 Randomized, Open-Label, Multicentre Study Evaluating the Efficacy of Axicabtagene Ciloleucel Versus Standard of Care Therapy in Subjects with Relapsed/Refractory Follicular Lymphoma	NCT05371093	Phase III	Recruiting	Oncology (Other)	Relapsed/Refractory Follicular Lymphoma	T cells	Yes, *ex vivo*	Retrovirus	Autologous	2023	University Hospital Birmingham NHS Foundation Trust, Birmingham University College London Hospitals NHS Foundation Trust, London The Royal Marsden NHS Foundation Trust, London

Cell and Gene Therapy Catapult ATMP Clinical Trials Database 2023

Sponsor	Title	Clinical Database Numbers	Trial Phase	Trial Status	Disease Area	Indication	Cell Type	Gene Modification / Gene Therapy	Type of Vector Used (if applicable)	Autologous/ Allogeneic	Year Trial Started	United Kingdom Site(s)
Kite Pharma Inc	A Phase 1 Open-label, Multicentre Study Evaluating the Safety and Efficacy of KITE-363, an Autologous Anti-CD19/CD20 CAR T-cell Therapy, in Subjects With Relapsed and/or Refractory B-cell Lymphoma	NCT04989803	Phase I	Recruiting	Oncology (Other)	Relapsed/Refractory Follicular Lymphoma	T cells	Yes, *ex vivo*	Retrovirus	Autologous	2023	King's College Hospital, London
Kite Pharma Inc	Study of Brexucabtagene Autoleucel (KTE-X19) in Participants With Relapsed/Refractory Mantle Cell Lymphoma	NCT04880434	Phase II	In follow-up	Oncology (Haematology)	Mantle Cell Lymphoma	T cells	Yes, *ex vivo*	Retrovirus	Autologous	2021	Queen Elizabeth University Hospital, Glasgow Kings College Hospital, London Manchester Royal Infirmary, Manchester
Kuopio University Hospital	Adenovirus Vascular Endothelial Growth Factor D (AdVEGF-D) Therapy for Treatment of Refractory Angina Pectoris (ReGenHeart)	NCT03039751	Phase II	Recruiting	Cardiovascular	Angina (Angina Pectoris) Coronary Artery Disease (CAD) (Ischemic Heart Disease) Refractory Angina	N/A	Yes, *in vivo*	Adenovirus	N/A	2019	Queen Mary University of London, London
Les Laboratoires Servier SAS	A Study to Evaluate the Long-term Safety of Patients with Advanced Lymphoid Leukemia Who Have Been Previously Administered with UCART19	NCT02735083	Phase I	In follow-up	Oncology (Haematology)	Advanced Lymphoid Malignancies	T cells	Yes, *ex vivo*	Lentivirus	Allogeneic	2021	The Christie NHS Foundation Trust, Manchester King's College Hospital NHS Foundation Trust, London Great Ormond Street Hospital for Children NHS Foundation Trust, London
Mario Negri Institute for Pharmacological Research	NEPHSTROM for Diabetic Kidney Disease (NEPHSTROM)	NCT02585622	Phase I/II	In follow-up	Renal and Urogenital	Type 2 Diabetes	Mesenchymal stem/stromal cells	No	N/A	Allogeneic	2017	Belfast Health and Social Care Trust - Belfast City Hospital, Belfast University Hospital Birmingham NHS Foundation Trust, Birmingham
MeiraGTx Holdings Plc	Long-Term Follow-up Gene Therapy Study for Leber Congenital Amaurosis OPTIRPE65 (Retinal Dystrophy Associated with Defects in RPE65)	NCT02946879	Phase I/II	Completed	Ophthalmology	Leber Congenital Amaurosis (LCA)	N/A	Yes, *in vivo*	AAV	N/A	2016	Moorfields Eye Hospital NHS Foundation Trust, London
MeiraGTx Holdings Plc	Long-term Follow-up Gene Therapy Study for Achromatopsia CNGB3 and CNGA3	NCT03278873	Phase I/II	In follow-up	Ophthalmology	Achromatopsia	N/A	Yes, *in vivo*	AAV	N/A	2017	Moorfields Eye Hospital NHS Foundation Trust, London

Cell and Gene Therapy Catapult ATMP Clinical Trials Database 2023

Sponsor	Title	Clinical Database Numbers	Trial Phase	Trial Status	Disease Area	Indication	Cell Type	Gene Modification / Gene Therapy	Type of Vector Used (if applicable)	Autologous/ Allogeneic	Year Trial Started	United Kingdom Site(s)
MeiraGTx Holdings Plc	Long Term Follow-Up Gene Therapy Study for XLRP RPGR	EudraCT-2018-000425-31	Phase I/II	Recruiting	Ophthalmology	X-Linked Retinitis Pigmentosa	N/A	Yes, *in vivo*	AAV	N/A	2018	Moorfields Eye Hospital NHS Foundation Trust, London The Leeds Teaching Hospitals NHS Trust, Leeds
MeiraGTx Holdings Plc	Gene Therapy Trial for the Treatment of X-linked Retinitis Pigmentosa Associated With Variants in the RPGR Gene	NCT04671433	Phase III	Recruiting	Ophthalmology	X-Linked Retinitis Pigmentosa	N/A	Yes, *in vivo*	AAV	N/A	2021	NHS Lothian, Edinburgh Glasgow Caledonian University, Glasgow Moorfields Eye Hospital NHS Foundation Trust, London St James's University Hospital, Leeds
MeiraGTx Holdings Plc	Follow-up Gene Therapy Trial for the Treatment of X-linked Retinitis Pigmentosa Associated with Variants in the RPGR Gene	NCT04794101	Phase III	Recruiting	Ophthalmology	X-Linked Retinitis Pigmentosa	N/A	Yes, *in vivo*	AAV	N/A	2021	NHS Lothian, Edinburgh Gartnavel General Hospital, Glasgow St Jame's University Hospital, Leeds Moorfields Eye Hospital NHS Foundation Trust, London
MeiraGTx Holdings Plc	A Study of Adeno-associated Virus Vector Serotype 8 (AAV8)- hCARp. Human Cyclic Nucleotide-gated Channel Beta 3 (hCNGB3) Gene Therapy for the Treatment of Achromatopsia Associated with Variants in the CNGB3 Gene	GDC30029380	Phase II	In planning/ set-up	Genetic Disorders	Achromatopsia	N/A	Yes, *in vivo*	AAV	N/A	2022	Moorfields Eye Hospital NHS Foundation Trust, London Manchester Royal Eye Hospital, Manchester"
Mina Therapeutics Ltd	First-in-human Safety and Tolerability Study of MTL-CEBPA in Patients with Advanced Liver Cancer (OUTREACH)	NCT02716012	Phase I	In follow-up	Oncology (Other)	Hepatocellular Carcinoma	N/A	Yes, *in vivo*	Non-viral	N/A	2016	University Hospitals Birmingham NHS Foundation Trust, Birmingham Cambridge University Hospitals NHS Trust, Cambridge The Beatson West of Scotland Cancer Centre, Glasgow Clatterbridge Cancer Centre NHS Foundation Trust, Liverpool Guy's and St. Thomas' NHS Foundation Trust, London Imperial College Healthcare NHS Trust, London University College London Hospital (UCLH), London Newcastle Upon Tyne Hospitals NHS Foundation Trust, Newcastle Upon Tyne
Moderna Therapeutics	A Study to Assess Safety, Pharmacokinetics, and Pharmacodynamics of mRNA-3705 in Participants With Isolated Methylmalonic Acidemia	NCT04899310	Phase I/II	Recruiting	Metabolic (Other)	Isolated Methylmalonic Acidemia Due to Methylmalonyl-CoA Mutase Deficiency	N/A	Yes, *in vivo*	Non-viral	N/A	2021	Royal Manchester Children's Hospital, Manchester Birmingham Childrens Hospital NHS Foundation Trust, Birmingham
Montpellier University Hospital	A Study Evaluating the Efficacy of a Single Injection Autologous Adipose Derived Mesenchymal Stromal Cells in Patients With Knee Osteoarthritis (ADIPOA-2) [[OR]] A Study Evaluating the Efficacy of a Single Injection Autologous Adipose Derived Mesenchymal Stromal Cells in Patients with Knee Osteoarthritis (ADIPOA-2)	NCT02838069	Phase II	In follow-up	Musculoskeletal	Osteoarthritis (knee)	Mesenchymal stem/stromal cells [[OR]] Adipose-derived stem cells	No	N/A	Autologous	2016	Cambridge University Hospitals NHS Foundation Trust, Cambridge

Cell and Gene Therapy Catapult ATMP Clinical Trials Database 2023

Sponsor	Title	Clinical Database Numbers	Trial Phase	Trial Status	Disease Area	Indication	Cell Type	Gene Modification / Gene Therapy	Type of Vector Used (if applicable)	Autologous/ Allogeneic	Year Trial Started	United Kingdom Site(s)
Moorfields Eye Hospital NHS Foundation Trust	A Study of Implantation of Retinal Pigment Epithelium in Subjects with Acute Wet Age Related Macular Degeneration	NCT01691261	Phase I	Recruiting	Ophthalmology	Acute Wet Age Related Macular Degeneration	Retinal	No	N/A	Allogeneic	2016	Moorfields Eye Hospital NHS Foundation Trust, London
Neurogene Inc	Gene Therapy Study for Children With CLN5 Batten Disease (CLN5-200)	GDC30028763	Phase I/II	Recruiting	Genetic Disorders	Batten Disease	N/A	Yes, *in vivo*	AAV	N/A	2022	Great Ormond Street Hospital for Children NHS Foundation Trust, London
NHS Blood and Transplant	Recovery and Survival of Stem Cell Originated Red Cells	ISRCTN42886452	Phase I	Recruiting	Haematological	Blood transfusions	CD34 and/or CD133 stem cells	No	N/A	Autologous	2018	NHS Blood and Transplant, (Cambridge Donor Centre), Cambridge
NHS Lothian	Cell Therapy for Acute Liver Injury Trial	ISRCTN12637839	Phase I	Recruiting	Gastrointestinal (Liver)	Acute Liver Injury	Monocytes	No	N/A	Allogeneic	2022	Edinburgh Royal Infirmary, Edinburgh
Novartis AG	CAR-T Long Term Follow Up (LTFU) Study (PAVO)	NCT02445222	Phase II/III	Recruiting	Oncology (Haematology)	Unspecified B-Cell Lymphomas B-Cell Leukemia	T cells	Yes, *ex vivo*	Lentivirus	Autologous	2020	Novartis Instigative Site, London
Novartis AG	Efficacy and Safety of Tisagenlecleucel in Adult Patients with Refractory or Relapsed Follicular Lymphoma (ELARA)	GDC30016008	Phase II	In follow-up	Oncology (Haematology)	Follicular Lymphoma	T cells	Yes, *ex vivo*	Lentivirus	Autologous	2018	Queen Elizabeth Hospital NHS Foundation Trust, London
Novartis AG	Tisagenlecleucel in Adult Patients with Aggressive B-cell Non-hodgkin Lymphoma (BELINDA)	GDC30016007	Phase III	In follow-up	Oncology (Haematology)	Non-Hodgkin Lymphoma	T cells	Yes, *ex vivo*	Lentivirus	Autologous	2014	Queen Elizabeth Hospital, Birmingham University College London Hospital (UCLH), London

Cell and Gene Therapy Catapult ATMP Clinical Trials Database 2023

Sponsor	Title	Clinical Database Numbers	Trial Phase	Trial Status	Disease Area	Indication	Cell Type	Gene Modification / Gene Therapy	Type of Vector Used (if applicable)	Autologous/ Allogeneic	Year Trial Started	United Kingdom Site(s)
Novartis AG	Phase II Open Label Trial to Determine Safety & Efficacy of Tisagenlecleucel in Pediatric Non-Hodgkin Lymphoma Patients (BIANCA)	GDC30018096	Phase II	Completed	Oncology (Haematology)	B-Cell Non-Hodgkin Lymphoma	T cells	Ye, *ex vivo*	Lentivirus	Autologous	2019	Royal Manchester Children's Hospital, Manchester
Novartis AG	Study of Efficacy and Safety of Tisagenlecleucel in HR B-ALL EOC MRD Positive Patients (CASSIOPEIA)	EudraCT-2017-002116-14	Phase II	Recruiting	Oncology (Haematology)	ALL, B-cell Leukaemia	T cells	Yes, *ex vivo*	Lentivirus	Autologous	2019	Novartis Investigative Site, London
Novartis AG	Safety and Efficacy of Intravenous OAV101 (AVXS-101) in Pediatric Patients with Spinal Muscular Atrophy (SMA) (SMART)	NCT04851873	Phase III	Completed	Neuromuscular	Spinal Muscular Atrophy	N/A	Yes, *in vivo*	AAV	N/A	2021	Great North Children's Hospital, Newcastle
Novartis AG	PHE885 CAR-T Therapy in Adult Participants with Relapsed and Refractory Multiple Myeloma	GDC30029597	Phase II	Recruiting	Oncology (Haematology)	Refractory Multiple Myeloma	T cells	Yes, *ex vivo*	Lentivirus	Autologous	2023	Novartis Investigative site, Birmingham Novartis Investigative site, Glasgow Novartis Investigative Site, Manchester
Novartis AG	Long-term Follow-up of Patients with Spinal Muscular Atrophy Treated with OAV101 in Clinical Trials	NCT05335876	Phase III	Recruiting	Neuromuscular	Spinal Muscular Atrophy	N/A	Yes, *in vivo*	AAV	N/A	2022	Newcastle Upon Tyne Hospitals NHS Foundation Trust, Newcastle Upon Tyne Novartis Investigative site, London
Novartis AG	CD19- and CD22-directed CAR-T Cell Therapy in Patients With Acute Lymphoblastic Leukemia	NCT05168748	Phase I	Withdrawn	Oncology (Haematology)	Acute Lymphocytic Leukemia	T cells	Yes, *ex vivo*	Lentivirus	Autologous	2023	Unconfirmed UK site(s)
Novartis Gene Therapies	Long-term Follow-up Study of Patients Receiving Onasemnogene Abeparvovec-xioi	NCT04042025	Phase III	In follow-up	Neuromuscular	Spinal Muscular Atrophy	N/A	Yes, *in vivo*	AAV	N/A	2020	Newcastle Upon Tyne Hospitals NHS Foundation Trust, Newcastle Upon Tyne

Cell and Gene Therapy Catapult ATMP Clinical Trials Database 2023

Sponsor	Title	Clinical Database Numbers	Trial Phase	Trial Status	Disease Area	Indication	Cell Type	Gene Modification / Gene Therapy	Type of Vector Used (if applicable)	Autologous/ Allogeneic	Year Trial Started	United Kingdom Site(s)
Ormond Medical Arts Pharmaceutical Research Center	Lentiviral Gene Therapy for p47 AR-CGD	NCT05207657	Phase I/II	Recruiting	Haematological	Chronic Granulomatous Disease	CD34 and/or CD133 stem cells	Yes, *ex vivo*	Lentivirus	Autologous	2022	Great Ormond Street Hospital for Children NHS Foundation Trust, London
Passage Bio Inc	Study of Safety, Tolerability and Efficacy of PBKR03 in Pediatric Subjects with Early Infantile Krabbe Disease (GALax-C)	GDC30020961	Phase I/II	In follow-up	Neurological	Krabbe Disease	N/A	Yes, *in vivo*	AAV	N/A	2022	Manchester University, Manchester
Passage Bio Inc	Study of Safety, Tolerability and Efficacy of PBGM01 in Pediatric Participants with GM1 Gangliosidosis (Imagine-1)	GDC30023951	Phase I/II	Recruiting	Metabolic (Other)	GM1 Gangliosidosis	N/A	Yes, *in vivo*	AAV	N/A	2021	Great Ormond Street Hospital for Children NHS Foundation Trust, London
Passage Bio Inc	A Study of PBFT02 in Patients with Frontotemporal Dementia and Progranulin Mutations (FTD-GRN) (upliFT-D)	GDC30025261	Phase I/II	Recruiting	Neurological	Frontotemporal Dementia	N/A	Yes, *in vivo*	AAV	N/A	2021	University of Cambridge, Cambridge
Pfizer Inc	Study to Evaluate the Safety and Efficacy of PF-06939926 for the Treatment of Duchenne Muscular Dystrophy	GDC30021209	Phase III	In follow-up	Neuromuscular	Duchenne Muscular Dystrophy	N/A	Yes, *in vivo*	AAV	N/A	2020	Great Ormond Street Hospital for Children NHS Foundation Trust, London Royal Liverpool and Broadgreen University Hospitals NHS Trust, Liverpool Royal Victoria Infirmary, Newcastle Upon Tyne Alder Hey Childrens NHS Foundation Trust, Liverpool
Pfizer Inc	Phase III, Open-Label, Single-Arm Study to Evaluate the Efficacy and Safety of PF-07055480 (Recombinant AAV2/6 Human Factor VIII Gene Therapy) in Adult Male Participants with Moderately Severe to Severe Hemophilia A (FVIII:C=1%)	GDC30023861	Phase III	Recruiting	Haematological	Haemophilia A	N/A	Yes, *in vivo*	AAV	N/A	2020	Guy's and St Thomas' NHS Foundation Trust, London
Prevail Therapeutics Inc	Phase I/II Clinical Trial of PR001 in Infants With Type 2 Gaucher Disease (PROVIDE)	GDC30020954	Phase I/II	Recruiting	Metabolic (Other)	Lysosomal Storage Disorder (Type 2 Gaucher disease)	N/A	Yes, *in vivo*	AAV	N/A	2021	Manchester Centre for Genomic Medicine, Manchester

Cell and Gene Therapy Catapult ATMP Clinical Trials Database 2023

Sponsor	Title	Clinical Database Numbers	Trial Phase	Trial Status	Disease Area	Indication	Cell Type	Gene Modification / Gene Therapy	Type of Vector Used (if applicable)	Autologous/ Allogeneic	Year Trial Started	United Kingdom Site(s)
Prevail Therapeutics Inc	Phase I/II Clinical Trial of PR006 in Patients with Frontotemporal Dementia with Progranulin Mutations (FTD-GRN)(PROCLAIM)	GDC30022518	Phase I/II	Recruiting	Neurological	Frontotemporal Dementia	N/A	Yes, *in vivo*	AAV	N/A	2020	University College London Hospital (UCLH), London
ProKidney LLC	A Study of REACT in Subjects with Type 2 Diabetes Mellitus and Chronic Kidney Disease	NCT05099770	Phase III	Recruiting	Renal and Urogenital	Chronic kidney disease	Unknown	No	N/A	Autologous	2022	Royal London Hospital, London
Quell Therapeutics Ltd	Safety and Clinical Activity of QEL-001 in A2-mismatch Liver Transplant Patients (LIBERATE)	GDC30029051	Phase I/II	Recruiting	Gastrointestinal (Liver)	Liver transplant rejection	T cells	Yes, *ex vivo*	Lentivirus	Autologous	2022	Cambridge University Hospitals NHS Foundation Trust, Cambridge Royal Free London NHS Foundation Trust, London King's College Hospital NHS Foundation Trust, London
RegenxBio Inc	A First-in-human Study in Pediatric Patients with Ocular Cln2 Disease	GDC30032053	Phase I/II	Recruiting	Genetic Disorders	Batten Disease	N/A	Yes, *in vivo*	AAV	N/A	2023	Great Ormond Street Hospital for Children NHS Foundation Trust, London
ReNeuron Group Ltd.	Pilot Investigation of Stem Cells in Stroke (PISCES)	NCT01151124	Phase I	Completed	Cardiovascular	Stable Ischemic Stroke	Neural	Yes, *ex vivo*	Retrovirus	Allogeneic	2010	Southern General Hospital, Glasgow
ReNeuron Group Ltd.	Safety and Tolerability of hRPC in Retinitis Pigmentosa (hRPCRP)	NCT02464436	Phase I/II	In follow-up	Ophthalmology	Retinitis Pigmentosa (Retinitis)	Retinal	No	N/A	Allogeneic	2020	Oxford Eye Hospital, Oxford
Replimune Ltd	Study of RP1 Monotherapy and RP1 in Combination with Nivolumab (IGNYTE)	GDC30015439	Phase I/II	Recruiting	Oncology (Other)	Melanoma	N/A	Yes, *in vivo*	Oncolytic Virus	N/A	2019	The Clatterbridge Cancer Centre NHS Foundation Trust, Liverpool Oxford University Hospitals NHS Trust, Oxford University of Leeds Teaching Hospital, Leeds The Royal Marsden NHS Foundation Trust, London St James's Iniversity Hospital, Leeds The Beatson West of Scotland Cancer Center, Glasgow

Cell and Gene Therapy Catapult ATMP Clinical Trials Database 2023

Sponsor	Title	Clinical Database Numbers	Trial Phase	Trial Status	Disease Area	Indication	Cell Type	Gene Modification / Gene Therapy	Type of Vector Used (if applicable)	Autologous/ Allogeneic	Year Trial Started	United Kingdom Site(s)
Replimune Ltd	Study of RP2 Monotherapy and RP2 in Combination with Nivolumab in Patients with Solid Tumors	GDC30019211	Phase I	Recruiting	Oncology (Other)	Solid Tumor Triple-Negative Breast Cancer (TNBC)	N/A	Yes, *in vivo*	Oncolytic Virus	N/A	2019	The Clatterbridge Cancer Centre NHS Foundation Trust, Liverpool The Royal Marsden NHS Foundation Trust, London Churchill Hospital, Oxford The Royal Marsden NHS Foundation Trust, Sutton
Replimune Ltd	Study of RP3 Monotherapy and RP3 in Combination with Nivolumab in Patients with Solid Tumours	GDC30019214	Phase I	Recruiting	Oncology (Other)	Advanced Solid Tumor	N/A	Yes, *in vivo*	Oncolytic Virus	N/A	2020	The Clatterbridge Cancer Centre NHS Foundation Trust, Liverpool The Royal Marsden NHS Foundation Trust, London Churchill Hospital, Oxford, The Royal Marsden NHS Foundation Trust, Sutton
Replimune Ltd	RP2/RP3 in Combination With Atezolizumab and Bevacizumab for the Treatment of Patients With CRC	GDC30027916	Phase II	Recruiting	Oncology (Other)	Metastatic Colorectal Cancer	N/A	Yes, *in vivo*	Oncolytic Virus	N/A	2023	Oxford University Hospitals NHS Foundation Trust, Oxford
Replimune Ltd	Rp3 in Combination with 1l or 2l Therapy in Patients with Locally Advanced Unresectable or Metastatic HCC	GDC30031984	Phase II	In planning/ set-up	Oncology (Other)	Hepatocellular Carcinoma Metastatic Hepatocellular Carcinoma (HCC)	N/A	Yes, *in vivo*	Oncolytic Virus	N/A	2023	The Clatterbridge Cancer Centre NHS Foundation Trust, Liverpool Oxford University Hospitals NHS Trust, Oxford University of Leeds Teaching Hospital, Leeds Royal Free London NHS Foundation Trust, London Kings College Hospital, London
Rocket Pharmaceuticals Inc	A Clinical Trial to Evaluate the Safety and Efficacy of RP-L201 in Subjects with Leukocyte Adhesion Deficiency-I	GDC30019116	Phase I/II	In follow-up	Inflammatory and immune system	Leukocyte Adhesion Deficiency-I (LAD-I)	CD34 and/or CD133 stem cells	Yes, *ex vivo*	Lentivirus	Autologous	2020	Great Ormond Street Hospital for Children NHS Foundation Trust, London University College London Hospital (UCLH), London
Rocket Pharmaceuticals Inc	Lentiviral-mediated Gene Therapy for Pediatric Patients With Fanconi Anemia Subtype A	EudraCT-2018-002502-31	Phase II	In follow-up	Haematological	Fanconi Anemia	CD34 and/or CD133 stem cells	Yes, *ex vivo*	Lentivirus	Autologous	2019	Great Ormond Street Hospital for Children NHS Foundation Trust, London
Sangamo Therapeutics Inc	Dose-Ranging Study of ST-920, an AAV2/6 Human Alpha Galactosidase A Gene Therapy in Subjects With Fabry Disease (STAAR)	GDC30006383	Phase I/II	In follow-up	Metabolic (Other)	Fabry Disease	N/A	Yes, *in vivo*	AAV	N/A	2019	Queen Elizabeth NHS Foundation Trust, London Cambridge University Hospitals NHS Foundation Trust, Cambridge Royal Free London NHS Foundation Trust, London

Cell and Gene Therapy Catapult ATMP Clinical Trials Database 2023

Sponsor	Title	Clinical Database Numbers	Trial Phase	Trial Status	Disease Area	Indication	Cell Type	Gene Modification / Gene Therapy	Type of Vector Used (if applicable)	Autologous/ Allogeneic	Year Trial Started	United Kingdom Site(s)
Sangamo Therapeutics Inc	Safety and Tolerability Study of Chimeric Antigen Receptor T-Reg Cell Therapy in Living Donor Renal Transplant Recipients (STeadfast)	GDC30014723	Phase I/II	Recruiting	Inflammatory and immune system	Kidney Transplant Rejection	T cells	Yes, *ex vivo*	Lentivirus	Autologous	2021	Oxford University Hospitals NHS Foundation Trust, Oxford
Sangamo Therapeutics Inc	Long-Term Follow-up of Subjects Who Were Treated with ST-920	NCT05039866	Phase I/II	Recruiting	Metabolic (Other)	Fabry Disease	N/A	Yes, *in vivo*	AAV	N/A	2021	Cambridge University Hospitals NHS Foundation Trust, Cambridge
Sangamo Therapeutics Inc	Long-Term Follow-Up of TX200-TR101 (STEADFAST Long Term)	2022-002440-40	Phase II	Recruiting	Renal and Urogenital	Kidney Transplant Rejection	T cells	Yes, *ex vivo*	Lentivirus	Autologous	2023	Oxford University Hospitals NHS Foundation Trust, Oxford
Sarepta Therapeutics Inc	A Gene Transfer Therapy Study to Evaluate the Safety and Efficacy of Delandistrogene Moxeparvovec (SRP-9001) in Participants with Duchenne Muscular Dystrophy (DMD) (EMBARK)	GDC30018143	Phase III	In follow-up	Genetic Disorders	Duchenne Muscular Dystrophy	N/A	Yes, *in vivo*	AAV	N/A	2021	Oxford University Hospitals NHS Foundation Trust, Oxford Great Ormond Street Hospital for Children NHS Foundation Trust, London Newcastle Upon Tyne NHS Hospital Foundation Trust, Newcastle Upon Tyne
Scancell Holdings Plc	A Phase II, Multicenter, Open-label Study of SCIB1 in Patients with Advanced Unresectable Melanoma Receiving Pembrolizumab	GDC30009247	Phase II	Recruiting	Oncology (Other)	Advanced unresectable melanoma	N/A	Yes, *in vivo*	Non-viral	N/A	2019	Velindre University NHS Trust, Cardiff East and North Hertfordshire NHS Trust, Northwood Nottingham University Hospitals NHS Trust, Nottingham Oxford University Hospital NHS Foundation Trust, Oxford
Solid Biosciences Inc	Microdystrophin Gene Transfer Study in Adolescents and Children with DMD (IGNITE DMD)	GDC30012329	Phase I/II	In follow-up	Genetic Disorders	Duchenne Muscular Dystrophy	N/A	Yes, *in vivo*	AAV	N/A	2017	Newcastle Upon Tyne Hospitals NHS Foundation Trust, Newcastle Upon Tyne
SOTIO Biotech AS	BOXR1030 T Cells in Subjects with Advanced GPC3-positive Solid Tumors	NCT05120271	Phase I/II	Recruiting	Oncology (Other)	Solid tumours	T cells	Yes, *ex vivo*	Lentivirus	Autologous	2022	The Royal Marsden NHS Foundation Trust, London University College London Hospital (UCLH), London Cardiff and Vale University, Cardiff The Beatson Institue for Cancer Research, Glasgow

Cell and Gene Therapy Catapult ATMP Clinical Trials Database 2023

Sponsor	Title	Clinical Database Numbers	Trial Phase	Trial Status	Disease Area	Indication	Cell Type	Gene Modification / Gene Therapy	Type of Vector Used (if applicable)	Autologous/ Allogeneic	Year Trial Started	United Kingdom Site(s)
Spark Therapeutics Inc	A Gene Transfer Study for Late-Onset Pompe Disease (RESOLUTE)	GDC30018726	Phase I/II	In follow-up	Metabolic (Other)	Lysosomal Storage Disorder	N/A	Yes, *in vivo*	AAV	N/A	2020	Salford Royal NHS Foundation Trust, Salford The Royal Free London NHS Foundation Trust, London Queen Elizabeth Hospital, Birmingham
St. Jude Children's Research Hospital Inc	Dose-escalation Study of A Self Complementary Adeno-associated Viral Vector For Gene Transfer in Hemophilia B	NCT00979238	Phase I/II	In follow-up	Haematological	Hemophilia B (Factor IX Deficiency)	N/A	Yes, *in vivo*	AAV	N/A	2010	Royal Free London NHS Foundation Trust, London
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)	GDC50005749	Phase IV	Recruiting	Gastrointestinal (Other)	Crohn's Disease and Complex Perianal Fistulas	Mesenchymal stem/stromal cells	No	N/A	Allogeneic	2018	St Mark's Hospital, Northern General Hospital, London Sheffield Teaching Hospitals NHS Foundation Trust, Sheffield
TC BioPharm Ltd	ACHIEVE - Efficacy and Effectiveness of Adoptive Cellular therapy with Ex-vivo Expanded Allogeneic yd T-lymphocytes (TCB-008) for Patients with Refractory or Relapsed Acute Myeloid Leukaemia (AML) (ACHIEVE)	GDC50011438	Phase II/III	Recruiting	Oncology (Haematology)	Refactory/ Relapsed Acute Myeloid Leukaemia	T cells	No	N/A	Allogeneic	2022	Bristol Haematology and Oncology Centre, Bristol Cardiff and Vale University LHB, Cardiff Queen Elizabeth University Hospital, London Guy's and St Thomas' NHS Foundation Trust, London Kings College Hospital NHS Foundation Trust, London Royal Marsden NHS Foundation Trust, London
The Robert Jones & Agnes Hunt Orthopaedic Hospital NHS Foundation Trust	Autologous Stem Cells, Chondrocytes or the Two? (ASCOT)	ISRCTN98997175	Phase II	In follow-up	Musculoskeletal	Osteoarthritis	Chondrocytes	No	N/A	Autologous	2013	The Robert Jones and Agnes Hunt Orthopaedic Hospital NHS Foundation Trust, Oswestry
The University of Manchester Royal Manchester Children's Hospital	Gene Therapy with Modified Autologous Hematopoietic Stem Cells for Patients with Mucopolysaccharidosis Type IIIA	GDC40001081	Phase I/II	In follow-up	Metabolic (Other)	Mucopolysaccharidosis IIIA (MPS IIIA) (Sanfilippo Syndrome) - Lysosomal storage disease	CD34 and/or CD133 stem cells	Yes, *ex vivo*	Lentivirus	Autologous	2020	Manchester University Hospital NHS Foundation Trust, Manchester Royal Manchester Children's Hospital, Manchester Great Ormond Street Hospital for Children NHS Trust, London King's College Hospital NHS Foundation Trust, London University College London Hospital (UCLH), London University Hospitals Coventry and Warwickshire NHS Trust, Coventry
TiGenix NV	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 Participants With Crohn's Disease (CD) (ADMIRE-CD-II)	GDC40002982	Phase III	Completed	Gastrointestinal (Other)	Crohn's Disease	Adipose-derived stem cells	No	N/A	Allogeneic	2019	Glasgow Royal Infirmary, Glasgow Addenbrooke's Hospital, Cambridge Royal Devon And Exeter Hospital, Exeter Wythenshawe Hospital, Manchester St. Mark's Hospital, London Guy's and St Thomas' NHS Foundation Trust, London

Cell and Gene Therapy Catapult ATMP Clinical Trials Database 2023

Sponsor	Title	Clinical Database Numbers	Trial Phase	Trial Status	Disease Area	Indication	Cell Type	Gene Modification / Gene Therapy	Type of Vector Used (if applicable)	Autologous/ Allogeneic	Year Trial Started	United Kingdom Site(s)
T-Knife GmbH	A Phase I/II, First-in-Human, Open-Label, Accelerated-Titration, Two-Part Clinical Trial of TK-8001 in Patients With HLA-A*02:01 Genotype and Advanced-Stage/Metastatic MAGE-A1+ Solid Tumors (IMAG1NE)	GDC30028271	Phase I/II	Recruiting	Oncology (Other)	Solid Tumor	T cells	Yes, *ex vivo*	Lentivirus	Autologous	2022	The Christie NHS Foundation Trust, Manchester
Transgene SA	A Clinical Trial Evaluating TG4050 in Head and Neck Cancer	GDC30018610	Phase I	Recruiting	Oncology (Other)	Squamous cell carcinoma of the head and neck	N/A	Yes, *in vivo*	MVA	N/A	2019	The Clatterbridge Cancer Centre NHS Foundation Trust, Liverpool Aintree University Hospital NHS Foundation Trust, Liverpool University Hospital NHS Foundation Trust, Southampton
Transgene SA	Study of Intrahepatic Arterial Infusion of TG6002 in Combination with 5-FC in Patients with Metastatic Colorectal Cancer	GDC30020393	Phase I/II	Terminated	Oncology (Other)	Colorectal Neoplasms	T cells	Yes, *in vivo*	Oncolytic Virus	N/A	2019	St James's University Hospital, Leeds
Trizell Ltd	Efficacy & Safety of rAd-IFN Administered with Celecoxib & Gemcitabine in Patients with Malignant Pleural Mesothelioma (INFINITE)	NCT03710876	Phase III	In follow-up	Oncology (Other)	Malignant Pleural Mesothelioma	N/A	Yes, *in vivo*	Adenovirus	N/A	2019	The Beatson West of Scotland Cancer Centre, Glasgow Derriford Hospital, Plymouth The Royal Marsden NHS Foundation Trust, London Guy's and St Thomas' NHS Foundation Trust, London Wythenshawe Hospital UHSM, Manchester Churchill Hospital, Oxford Queen Elizabeth Hospital, Birmigham
Turnstone Biologics, Corp.	A Phase 1/2a, Multicenter, Open-label Trial of TBio-6517, an Oncolytic Vaccinia Virus, Administered by Intratumoral Injection, Alone and in Combination With Pembrolizumab, in Patients With Advanced Solid Tumors	2021-001001-79 NCT04301011 TBio-6517-ITu-001	Phase I/II	In follow-up	Oncology (Other)	Solid Tumors	N/A	Yes, *in vivo*	Oncolytic Virus	N/A	2022	Unknown UK site(s)
Ultragenyx Pharmaceutical Inc	Long Term Follow up to Evaluate DTX301 in Adults with Late-onset Otc Deficiency (CAPtivate)	EudraCT-2018-000156-18	Phase I/II	In follow-up	Metabolic (Other)	Ornithine-Transcarbamylase Deficiency	N/A	Yes, *in vivo*	AAV	N/A	2018	Queen Elizabeth Hospital, Birmingham
Ultragenyx Pharmaceutical Inc	Clinical Study of DTX301 AAV- Mediated Gene Transfer for Ornithine Transcarbamylase(OTC) Deficiency	GDC30021161	Phase III	Recruiting	Genetic Disorders	Ornithine-Transcarbamylase Deficiency	N/A	Yes, *in vivo*	AAV	N/A	2022	University Hospitals, Birmingham

Cell and Gene Therapy Catapult ATMP Clinical Trials Database 2023

Sponsor	Title	Clinical Database Numbers	Trial Phase	Trial Status	Disease Area	Indication	Cell Type	Gene Modification / Gene Therapy	Type of Vector Used (if applicable)	Autologous/ Allogeneic	Year Trial Started	United Kingdom Site(s)
Ultragenyx Pharmaceutical Inc	Study of UX701 Gene Transfer for the Treatment of Wilson Disease	GDC30026687	Phase II/III	Recruiting	Genetic Disorders	Wilson Disease	N/A	Yes, *in vivo*	AAV	N/A	2021	Kings College Hospital NHS Foundation Trust, London
UniQure NV	Safety and Efficacy of AMT-130 in European Adults With Early Manifest Huntington's Disease	GDC30027414	Phase I/II	Recruiting	Neurological	Huntington Disease	N/A	Yes, *in vivo*	AAV	N/A	2021	University Hospital of Wales, Cardiff University College London Hospital (UCLH), London
University College London	Immunotherapy With CD19/22 CAR Redirected T-cells for High Risk, Relapsed Paediatric CD19+ and/or CD22+ Acute Lymphoblastic Leukaemia and Other Haematological Malignancies	NCT02443831	Phase I	In follow-up	Oncology (Haematology)	Haematological Malignancies	T cells	Yes, *ex vivo*	Lentivirus	Autologous	2016	Royal Manchester Children's Hospital, Manchester Great Ormond Street Hospital for Children NHS Trust, London University College London Hospital (UCLH), London
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 (ALLCAR19)	NCT02935257	Phase I/II	Recruiting	Oncology (Haematology)	Acute Lymphocytic Leukemia	T cells	Yes, *ex vivo*	Lentivirus	Autologous	2017	University College London Hospital (UCLH), London The Christie NHS Foundation Trust, Manchester The Beatson West of Scotland Cancer Centre / Queen Elizabeth University Hospital, Glasgow
University College London	Gene Therapy for Haemophilia A (GO-8)	NCT03001830	Phase I/II	Recruiting	Haematological	Hemophilia A (Factor VIII Deficiency)	N/A	Yes, *in vivo*	AAV	N/A	2017	Royal Free London NHS Foundation Trust, London
University College London	Immunotherapy with Tacrolimus Resistant EBV CTL for Lymphoproliferative Disease after Solid Organ Transplant (ITREC)	NCT03131934	Phase I	In follow-up	Oncology (Haematology)	Post-Transplant Lymphoproliferative Disorder	T cells	Yes, *ex vivo*	Retrovirus	Autologous	2019	Great Ormond Street Hospital for Children NHS Foundation Trust, London Kings College Hospital NHS Foundation Trust, London
University College London	Targeted Stem Cells Expressing Trail as a Therapy for Lung Cancer	NCT03298763	Phase I/II	Recruiting	Oncology (Other)	Non-Small Cell Lung Cancer	Mesenchymal stem/stromal cells	Yes, *ex vivo*	Lentivirus	Allogeneic	2019	University College London Hospital (UCLH), London

Cell and Gene Therapy Catapult ATMP Clinical Trials Database 2023

Sponsor	Title	Clinical Database Numbers	Trial Phase	Trial Status	Disease Area	Indication	Cell Type	Gene Modification / Gene Therapy	Type of Vector Used (if applicable)	Autologous/ Allogeneic	Year Trial Started	United Kingdom Site(s)
University College London	Transfer of Effector Memory T Cells (Tem) Following Allogeneic Stem Cell Transplantation (ToTem)	NCT03836690	Phase I	Recruiting	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)	T cells	No	N/A	Allogeneic	2019	University College London Hospital (UCLH), London
University College London	A New Study Evaluating the Activity of Modular CAR T for Myeloma (MCARTY)	GDC20027267	Phase I	Recruiting	Oncology (Haematology)	Multiple Myeloma	T cells	Yes, *ex vivo*	Lentivirus	Autologous	2022	University College London Hospital (UCLH), London
University College London	Immunotherapy Using CAR T-cells to Target CD19 for Relapsed/Refractory CD19+ Primary CNS Lymphoma (CAROUSEL)	NCT04443829	Phase I	In follow-up	Oncology (Other)	Primary CNS Lymphoma	T cells	Yes, *ex vivo*	Unknown	Autologous	2021	University College London Hospital (UCLH), London
University College London	Halting Ornithine Transcarbamylase Deficiency With Recombinant AAV in Children (HORACE)	NCT05092685	Phase I/II	Recruiting	Genetic Disorders	Ornithine-Transcarbamylase Deficiency	N/A	Yes, *in vivo*	AAV	N/A	2023	Great Ormond Street Hospital for Children NHS Foundation Trust, London
University of Birmingham	A Single-arm, Phase I/IIa, Safety and Efficacy Trial of Selected MSCs in the Treatment of Patients with PSC and AiH (Merlin)	NCT02997878	Phase II	In follow-up	Gastrointestinal (Liver)	Primary Sclerosing Cholangitis (PSC)	Mesenchymal stem/stromal cells	No	N/A	Allogeneic	2018	Queen Elizabeth Hospital, Birmingham University Hospitals, Birmingham
University of Birmingham	A single arm, multi-centre, phase IIa basket trial investigating the safety and activity of the use of ORBCEL-C™ in the treatment of patients with Primary Sclerosing Cholangitis, Rheumatoid Arthritis and Crohn's Disease - POLARISE	IRAS-272535	Phase II	In planning/ set-up	Gastrointestinal (Liver)	Lupus nephritis, PSC, Rheumatoid arthitis, crohns disease	Mesenchymal stem/stromal cells	No	N/A	Allogeneic	2020	University Hospitals, Birmingham
University of Birmingham	A Global Study of Novel Agents in Paediatric and Adolescent Relapsed and Refractory B-cell Non-Hodgkin Lymphoma (Glo-BNHL)	GDC50013300	Phase II/III	In planning/ set-up	Oncology (other)	B-Cell Non-Hodgkin Lymphoma	T cells	Yes, *ex vivo*	Lentivirus	Autologous	2023	Unconfirmed UK Site(s)

Cell and Gene Therapy Catapult ATMP Clinical Trials Database 2023

Sponsor	Title	Clinical Database Numbers	Trial Phase	Trial Status	Disease Area	Indication	Cell Type	Gene Modification / Gene Therapy	Type of Vector Used (if applicable)	Autologous/ Allogeneic	Year Trial Started	United Kingdom Site(s)
University of Birmingham	A Clinical Trial to See If a Mesenchymal Stem Cells Treatment Called Orbcel-C Can Help to Treat Primary Sclerosing Cholangitis, Rheumatoid Arthritis, Lupus Nephritis and Crohn's Disease	ISRCTN80103507	Phase II	Recruiting	Gastrointestinal (Other)	Primary Sclerosing Cholangitis Rheumatoid Arthritis Lupus Nephritis Crohn's Disease (Regional Enteritis)	Mesenchymal stem/stromal cells	No	N/A	Allogeneic	2018	Queen Elizabeth Hospital, Birmingham Queens Medical Centre, Nottingham University Hospital, Nottingham
University of California Los Angeles	An Observational LTFU Study for Patients Previously Treated with Autologous ex Vivo Gene Therapy for ADA-SCID	NCT04049084	Phase III	Recruiting	Inflammatory and immune system	Adenosine Deaminase (ADA) Deficiency Related SCID	CD34 and/or CD133 stem cells	Yes, *ex vivo*	Lentivirus	Autologous	2019	UCL Great Ormond Street Institute of Child Health, London
University of Oxford	TWO study: Cell Therapy Trial in Renal Transplantation	ISRCTN11038572	Phase II	Recruiting	Inflammatory and immune system	Kidney Transplant Rejection	T cells	No	N/A	Autologous	2018	University of Oxford, Oxford
Vertex Pharmaceuticals Inc	A Safety and Efficacy Study Evaluating CTX001 in Subjects with Transfusion-dependent ß-thalassemia	GDC30015188	Phase I/II	In follow-up	Haematological	Transfusion-Dependent β Thalassemia	CD34 and/or CD133 stem cells	Yes, *ex vivo*	Non-viral	Autologous	2018	Imperial College Healthcare NHS Trust, London University College London Hospital (UCLH), London
Vertex Pharmaceuticals Inc	A Safety and Efficacy Study Evaluating CTX001 in Subjects With Severe Sickle Cell Disease	GDC30016262	Phase II/III	In follow-up	Haematological	Severe Sickle Cell Disease (SCD)	CD34 and/or CD133 stem cells	Yes, *ex vivo*	Non-viral	Autologous	2018	Imperial College Healthcare NHS Trust, London Royal London and St Bartholomews Hospital, London
Vertex Pharmaceuticals Inc	A Long-term Follow-up Study in Subjects Who Received CTX001	NCT04208529	Phase II	Recruiting	Haematological	Multiple	CD34 and/or CD133 stem cells	Yes, *ex vivo*	Non-viral	Autologous	2021	Imperial College Healthcare NHS Trust, London Hammersmith Hospital, London
Vertex Pharmaceuticals Inc	Evaluation of Safety and Efficacy of CTX001 in Pediatric Participants With Transfusion-Dependent ß-Thalassemia (TDT)	EudraCT-2021-002172-39	Phase III	Recruiting	Genetic Disorders	Transfusion-Dependent β-Thalassemia	CD34 and/or CD133 stem cells	Yes, *ex vivo*	N/A	Autologous	2022	Great Ormond Street Hospital for Children NHS Foundation Trust, London St Mary's Hospital, London

Cell and Gene Therapy Catapult ATMP Clinical Trials Database 2023

Sponsor	Title	Clinical Database Numbers	Trial Phase	Trial Status	Disease Area	Indication	Cell Type	Gene Modification / Gene Therapy	Type of Vector Used (if applicable)	Autologous/ Allogeneic	Year Trial Started	United Kingdom Site(s)
Vertex Pharmaceuticals Inc	Evaluation of Safety and Efficacy of CTX001 in Pediatric Participants With Severe Sickle Cell Disease (SCD)	EudraCT-2021-002173-26	Phase III	Recruiting	Haematological	Severe Sickle Cell Disease (SCD)	CD34 and/or CD133 stem cells	Yes, *ex vivo*	N/A	Autologous	2022	St Mary's Hospital, London
Videregen Ltd	Engineered Tissue Repair of Bronchopleural Fistula (ENTRUST)	NCT04435249	Phase I/II	In planning/ set-up	Respiratory	Bronchopleural Fistula	Mesenchymal stem/stromal cells	No	N/A	Autologous	2023	Royal Papworth Hospital, Cambridge Royal Free London NHS Foundation Trust, London
Vivet Therapeutics SAS	A Phase I/II Study of VTX-801 in Adult Patients with Wilson's Disease (GATEWAY)	GDC30013710	Phase I/II	Recruiting	Metabolic (Other)	Wilson Disease	N/A	Yes, *in vivo*	AAV	N/A	2021	Royal Surrey County Hospital, Guildford