Cell and Gene Therapy Catapult ATMP Clinical Trials Database 2021

Sponsor	Title	Clinical database Numbers	United Kingdom Site(s)	Status	Phase	Year Started	Cell type	Gene modification / gene therapy	Type of Vector Used (if applicable)	Autologous/ Allogeneic	Disease Area	Indication
Abeona Therapeutics Inc	Phase I/II Gene Transfer Clinical Trial of rAAV9.CMV.hNAGLU for Mucopolysaccharidosis (MPS) IIIB	NCT03315182 EudraCT-2014-001411-39	Unknown UK site(s)	Withdrawn	Phase I/II	2019	N/A	Yes, in vivo	AAV	N/A	Metabolic (Other)	Mucopolysaccharidosis III (MPS III)
Achilles Therapeutics UK Ltd	An Open-label, Multi-Centre Phase I/IIa Study Evaluating the Safety and Clinical Activity of Neoantigen Reactive T Cells in Patients with Metastatic or Recurrent Melanoma	NCT03997474 EudraCT-2018-003446-16	The Christie NHS Foundation Trust, Manchester Freeman Hospital, Newcastle upon Tyne Cambridge University Hospitals NHS Foundation Trust University College London Hospitals NHS Foundation Trust Guy's and St Thomas' NHS Foundation Trust, London The Royal Marsden NHS Foundation Trust, London University Hospital Southampton NHS Foundation Trust	Recruiting	Phase I/II	2019	T cells	No	N/A	Autologous	Oncology (Other)	Metastatic or Recurrent Melanoma
Achilles Therapeutics UK Ltd	An Open-Label, Multi-Centre Phase I/IIa Study Evaluating the Safety and Clinical Activity of Neoantigen Reactive T Cells in Patients with Advanced Non-Small Cell Lung Cancer	NCT04032847 EudraCT-2018-001005-85	University Hospitals Birmingham NHS Foundation Trust Cambridge University Hospitals NHS Foundation Trust The Christie NHS Foundation Trust, Manchester University College London Hospitals NHS Foundation Trust University Hospital Southampton NHS Foundation Trust Freeman Hospital, Newcastle upon Tyne Leeds Teaching Hospitals NHS Trust Guy's and St Thomas' NHS Foundation Trust, London	Recruiting	Phase I/II	2019	T cells	No	N/A	Autologous	Oncology (Other)	Non-Small Cell Lung Cancer
Achilles Therapeutics UK Ltd	An Open-Label, Multi-Centre Phase II Study Evaluating the Long-Term Safety and Clinical Activity of Neoantigen Reactive T Cells in Patients Who Have Previously Received ATL001 in a Clinical Trial	NCT04785365	University Hospitals Birmingham NHS Foundation Trust Cambridge University Hospitals NHS Foundation Trust Leeds Teaching Hospitals NHS Trust University College London Hospitals NHS Foundation Trust 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	In planning/set-up	Phase II	2022	T cells	No	N/A	Autologous	Oncology (Other)	Metastatic or Recurrent Melanoma / Non-Small Cell Lung Cancer
Adaptimmune Therapeutics Plc	A Phase I Dose Escalation Open Label Clinical Trial Evaluating the Safety and Efficacy of MAGE A10^{c796}T in Subjects With Stage IIIb or Stage IV Non-Small Cell Lung Cancer (NSCLC)	NCT02592577 EudraCT-2016-002518-28	University College Hospital Macmillan Cancer Centre The Christie NHS Foundation Trust, Manchester	Completed	Phase I	2015	T cells	Yes, ex vivo	Lentivirus	Autologous	Oncology (Other)	Non-Small Cell Lung Cancer
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 (AFP^{c332}T) 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, London University College London Hospitals NHS Foundation Trust The Christie NHS Foundation Trust, Manchester	Recruiting	Phase I	2017	T cells	Yes, ex vivo	Lentivirus	Autologous	Oncology (Other)	Hepatocellular Carcinoma Liver Cancer
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 EudraCT-2019-000589-39	University College London Hospitals NHS Foundation Trust 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

Cell and Gene Therapy Catapult ATMP Clinical Trials Database 2021

Sponsor	Title	Clinical database Numbers	United Kingdom Site(s)	Status	Phase	Year Started	Cell type	Gene modification / gene therapy	Type of Vector Used (if applicable)	Autologous/ Allogeneic	Disease Area	Indication
Adaptimmune Therapeutics Plc	A Phase II Open-Label Clinical Trial of ADP-A2M4CD8 in Subjects With Advanced Esophageal or Esophagogastric Junction Cancers	NCT04752358 EudraCT-2020-005802-24	The Christie NHS Foundation Trust, Manchester Beatson West of Scotland Cancer Centre University College London Hospitals NHS Foundation Trust Guy's and St Thomas' NHS Foundation Trust, London	In planning/set-up	Phase II	2021	T cells	Yes, ex vivo	Lentivirus	Autologous	Oncology (Other)	Oesophageal Cancer
AlloVir Inc	Phase III Multicenter, Double-blind, Placebo-controlled Trial of Viralym-M (ALVR105) for the Treatment of Patients with Virus-associated Hemorrhagic Cystitis after Allogeneic Hematopoietic Cell Transplant (HCT)	NCT04390113 EudraCT-2020-000722-26	Unknown UK site(s)	In planning/set-up	Phase III	2021	T cells	No	N/A	Allogeneic	Renal and Urogenital	Hemorrhagic Cystitis
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	NCT04693637 2021-003450-22	Unknown UK site(s)	In planning/set-up	Phase II	2021	T cells	No	N/A	Allogeneic	Infectious Disease	High-Risk Allogeneic Hematopoietic Cell Transplant patients
Amgen Inc	A Phase Ib/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 EudraCT-2014-000185-22	Research Site, Birmingham, B15 2TH Research Site, Guildford, GU2 7XX Research Site, Leeds, LS9 7TF Research Site, Leicester, LE1 5WW Research Site, London, SE1 9RT Research Site, London, SW3 6JJ Research Site, Manchester, M20 4BX Research Site, Oxford, OX3 7LJ Research Site, Preston, PR2 9HT Research Site, Southampton, SO16 6YD	Terminated	Phase III	2014	N/A	Yes, in vivo	Oncolytic Virus	N/A	Oncology (Other)	Melanoma
Amgen Inc	A Registry Study to Evaluate the Survival and Long-term Safety of Subjects who Previously Received Talimogene Laherparepvec in Amgen or BioVEX-Sponsored Clinical Trials	NCT02173171 EudraCT-2015-003196-29	Guy's and St Thomas' NHS Foundation Trust, London The Royal Marsden NHS Foundation Trust, London The Clatterbridge Cancer Centre NHS Foundation Trust	Completed	Phase IV	2018	N/A	Yes, in vivo	Oncolytic Virus	N/A	Oncology (Other)	Various cancers
Amgen Inc	A Phase II, Multicenter, Open-label, Single-arm Trial to Evaluate the Correlation Between Objective Response Rate and Baseline Intratumoral CD8+ Cell Density in Subjects with Unresected Stage IIIB to IVM1c Melanoma Treated with Talimogene Laherparepvec	NCT02366195 EudraCT-2013-005552-15	Research Site, London, SE1 9RT Research Site, Wirral, CH63 4JY	Completed	Phase II	2015	N/A	Yes, in vivo	Oncolytic Virus	N/A	Oncology (Other)	Melanoma
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 EudraCT-2015-003011-38	Research Site, Birmingham, B15 2TT Research Site, London, SE1 7EH Research Site, London, SW3 6JJ Research Site, Oxford, OX3 7LE Research Site, Sutton, SM2 5PT Research Site, Wirral, CH63 4JY	Completed	Phase III	2016	N/A	Yes, in vivo	Oncolytic Virus	N/A	Oncology (Other)	Carcinoma of the Head and Neck

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Sponsor	Title	Clinical database Numbers	United Kingdom Site(s)	Status	Phase	Year Started	Cell type	Gene modification / gene therapy	Type of Vector Used (if applicable)	Autologous/ Allogeneic	Disease Area	Indication
Amgen Inc	A Postmarketing Prospective Cohort Study of Melanoma Patients Treated with IMLYGIC® in Clinical Practice to Characterize Risk of Herpetic Infection Among Patients, Close Contacts, Health Care Providers & Long-term Safety in Treated Patients	NCT02910557 EUPAS15128	Cheltenham General Hospital, Cheltenham Churchill Hospital, Oxford	Recruiting	Phase IV	2017	N/A	Yes, in vivo	Oncolytic Virus	N/A	Oncology (Other)	Melanoma
Amgen Inc	Phase II Study of Talimogene Laherparepvec in Combination with Pembrolizumab in Subjects with Unresectable/Metastatic Stage IIIB-IVM1d Melanoma who have Progressed on Prior Anti-PD-1 Based Therapy	NCT04068181 EudraCT-2019-001906-61	Guy's and St Thomas' NHS Foundation Trust, London The Royal Marsden NHS Foundation Trust, London	In follow-up	Phase II	2020	N/A	Yes, in vivo	Oncolytic Virus	N/A	Oncology (Other)	Melanoma
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 EudraCT-2017-001774-41	University of Leicester University Hospitals Birmingham NHS Foundation Trust	Recruiting	Phase II/III	2018	Antigen presenting cells	No	N/A	Autologous	Oncology (Other)	Malignant Pleural Mesothelioma Malignant Mesothelioma
Astellas Gene Therapies	VALENS: 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	NCT03223194 EudraCT-2017-001631-39	King's College Hospital NHS Foundation Trust, London	Terminated	Phase I/II	2017	N/A	Yes, in vivo	AAV	N/A	Metabolic (Other)	Crigler-Najjar Syndrome
Astellas Gene Therapies	ASPIRO: A Phase I/II, 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 EudraCT-2017-000876-27	Great Ormond Street Hospital NHS Foundation Trust, London	In follow-up	Phase I/II	2017	N/A	Yes, in vivo	AAV	N/A	Neuromuscular	X-Linked Myotubular Myopathy (XLMTM or MTM)
Astellas Gene Therapies	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 EudraCT-2019-003595-38	The Newcastle upon Tyne Hospitals NHS Foundation Trust	Recruiting	Phase I/II	2020	N/A	Yes, in vivo	AAV	N/A	Metabolic (Other)	Pompe Disease
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 EudraCT-2016-005245-23	Site GB44001, London, United Kingdom	Recruiting	Phase I/II	2018	Retinal	No	N/A	Allogeneic	Ophthalmology	Age Related Macular Degeneration Dry (Atrophic) Macular Degeneration
AstraZeneca Plc	An Open-label Phase I Study to Assess the Safety, Tolerability, Pharmacokinetics, Pharmacodynamics and Preliminary Efficacy of MEDI5395 in Combination with Durvalumab in Subjects with Select Advanced Solid Tumors	NCT03889275	Research Site, Leeds, LS9 7TF Research Site, London, SW3 6JJ	In follow-up	Phase I	2019	N/A	Yes, in vivo	Oncolytic Virus	N/A	Oncology (Other)	Advanced Solid Tumors

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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 EudraCT-2017-002949-30	Cambridge University Hospitals NHS Foundation Trust	Recruiting	Phase III	2020	T cells	No	N/A	Allogeneic	Oncology (Other)	Epstein-Barr Virus-Associated Post-Transplant Lymphoproliferative Disease
Athersys Inc	MultiStem® Administration for Stroke Treatment and Enhanced Recovery Study (MASTERS-2)	NCT03545607 EudraCT-2019-001680-69	Unknown UK site(s)	In planning/set-up	Phase III	2021	Mesenchymal stem / stromal cells	No	N/A	Allogeneic	Cardiovascular	Acute Ischaemic Stroke
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 EudraCT-2017-001965-26	The Christie NHS Foundation Trust, Manchester Freeman Hospital, Newcastle upon Tyne Manchester Royal Infirmary University College London Hospitals NHS Foundation Trust Beatson West of Scotland Cancer Centre / Queen Elizabeth University Hospital	Recruiting	Phase I/II	2018	T cells	Yes, ex vivo	Retrovirus	Autologous	Oncology (Haematology)	T-Cell Lymphomas
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 EudraCT-2016-004682-11	Freeman Hospital, Newcastle upon Tyne The Christie NHS Foundation Trust, Manchester University College London Hospitals NHS Foundation Trust Beatson West of Scotland Cancer Centre / Queen Elizabeth University Hospital	Recruiting	Phase I/II	2017	T cells	Yes, ex vivo	Retrovirus	Autologous	Oncology (Haematology)	B-Cell lymphoma
Autolus Therapeutics Plc	Long-term Follow-up of Patients Previously Treated with Autologous T Cells Genetically Modified with Viral Vectors	NCT03628612 EudraCT-2016-004867-38	Great Ormond Street Hospital NHS Foundation Trust, London University College London Hospitals NHS Foundation Trust Royal Manchester Children's Hospital, Manchester Freeman Hospital, Newcastle upon Tyne	Recruiting	Phase II	2018	T cells	Yes, ex vivo	Retrovirus	Autologous	Oncology (Other)	Multiple Myeloma
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 EudraCT-2019-001937-16	University Hospitals Bristol and Weston NHS Foundation Trust Queen Elizabeth University Hospital, Glasgow University College London Hospitals NHS Foundation Trust King's College Hospital NHS Foundation Trust, London Manchester Royal Infirmary Freeman Hospital, Newcastle upon Tyne Beatson West of Scotland Cancer Center, Glasgow	Recruiting	Phase I/II	2020	T cells	Yes, ex vivo	Lentivirus	Autologous	Oncology (Haematology)	B-Cell Acute Lymphocytic Leukemia
Azellon Ltd	A Prospective Open-label Study to Evaluate the Safety of Cell Bandage (Mesenchymal Stem Cells) in the Treatment of Meniscal Tears	EudraCT-2010-024162-22	Southmead Hospital, Bristol	Completed	Phase I/II	2012	Mesenchymal stem/stromal cells	No	N/A	Autologous	Musculoskeletal	Knee Meniscus Repair
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 EudraCT-2018-001063-23	St Bartholomew's Hospital, London	Recruiting	Phase II	2018	Bone marrow mononuclear cells	No	N/A	Autologous	Cardiovascular	Dilated Cardiomyopathy

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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 EudraCT-2017-000806-39	St Thomas' Hospital London, Manchester Royal Infirmary	In follow-up	Phase I/II	2018	N/A	Yes, in vivo	AAV	N/A	Haematological	Hemophilia A (Factor VIII Deficiency)
Belfast Health and Social Care Trust	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 EudraCT-2017-000584-33	Royal Hospitals, Belfast Health and Social Care Trust Guy's and St Thomas' NHS Foundation Trust, London King's College Hospital, King's College Hospital NHS Foundation Trust, London University Hospitals Birmingham NHS Foundation Trust Edinburgh Royal Infirmary, Royal Infirmary of Edinburgh University College London Hospitals NHS Foundation Trust Manchester Royal Infirmary Sunderland Hospital, South Tyneside NHS Foundation Trust Liverpool Royal Infirmary, Liverpool University Hospitals NHS Foundation Trust University Hospital Southampton NHS Foundation Trust Wythenshawe Hospital, Manchester University NHS Foundation Trust Aintree University Hospital, Liverpool University Hospitals NHS Foundation Trust	In follow-up	Phase I/II	2018	Mesenchymal stem/stromal cells	No	N/A	Allogeneic	Respiratory	Acute Respiratory Distress Syndrome (ARDS)
Bellicum Pharmaceuticals Inc	Phase I/II study of CaspaCIDe T cells (BPX-501) from an HLA-partially matched family donor after negative selection of TCR αβ+T cells in pediatric patients affected by hematological disorders	NCT02065869 EudraCT-2014-000584-41	Great Ormond Street Hospital NHS Foundation Trust, London Great North Children's Hospital Newcastle Upon Tyne Royal Free London NHS Foundation Trust	Recruiting	Phase I/II	2015	T cells	Yes, ex vivo	Retrovirus	Allogeneic	Oncology (Haematology)	Haematological Malignancies
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 EudraCT-2015-003958-41	Manchester Royal Infirmary John Radcliffe Hospital Oxford	Completed	Phase III	2016	N/A	Yes, in vivo	AAV	N/A	Ophthalmology	Choroideremia
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 EudraCT-2016-003852-60	Manchester Royal Eye Hospital, Manchester Oxford Eye Hospital Study Site, Southampton	Completed	Phase I/II/III	2017	N/A	Yes, in vivo	AAV	N/A	Ophthalmology	Retinitis Pigmentosa (Retinitis)

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Biogen Inc	A Long-term Follow-up Study to Evaluate the Safety and Efficacy of Retinal Gene Therapy in Subjects with Choroideremia Previously Treated with Adeno-Associated Viral Vector Encoding Rab Escort Protein-1 (AAV2-REP1) and in Subjects with X-Linked Retinitis Pigmentosa Previously Treated with Adeno-Associated Viral Vector Encoding RPGR (AAV8-RPGR) in an Antecedent Study	NCT03584165 EudraCT-2017-003104-42	Research Site London, EC1V 2PD Research Site Manchester, M13 9WL Research Site Oxford	Recruiting	Phase III	2018	N/A	Yes, in vivo	AAV	N/A	Ophthalmology	Choroideremia
BioMarin Pharmaceutical Inc	A Phase I/II, Dose-escalation Safety, Tolerability and Efficacy Study of Valoctocogene Roxaparvovec, an Adenovirus-Associated Virus Vector-Mediated Gene Transfer of Human Factor VIII in Patients with Severe Haemophilia A	NCT02576795 EudraCT-2014-003880-38	Hammersmith Hospital, London The Royal London Hospital Basingstoke and North Hampshire Hospital, Basingstoke Queen Elizabeth Hospital, Birmingham Guy's and St. Thomas' NHS Foundation Trust, London Royal Infirmary, Glasgow Addenbrooke's Hospital, Cambridge Bristol Haematology and Oncology Centre University Hospital Southampton NHS Foundation Trust	In follow-up	Phase I/II	2015	N/A	Yes, in vivo	AAV	N/A	Haematological	Hemophilia A (Factor VIII Deficiency)
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 <= 1 IU/dL Receiving Prophylactic FVIII Infusions	NCT03370913 EudraCT-2017-003215-19	Queen Elizabeth Hospital, Birmingham Addenbrooke's Hospital, Cambridge Glasgow Royal Infirmary, Glasgow Barts and The London School of Medicine and Dentistry, London Hammersmith Hospital London 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
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 at a Dose of 4E13vg/kg in Hemophilia A Patients With Residual FVIII Levels <=1IU/dL Receiving Prophylactic FVIII Infusions	NCT03392974 EudraCT-2017-003573-34	Addenbrookes Hospital, Cambridge Glasgow Royal Infirmary Barts and The London School of Medicine and Dentistry, London Hammersmith Hospital, London St Thomas' Hospital, London Churchill Hospital, Oxford University Hospital Southampton NHS Foundation Trust Queen Elizabeth Hospital, Birmingham	Withdrawn	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 Roxaparvovec, 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 EudraCT-2017-000662-29	Royal Free London NHS Foundation Trust 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)

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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 EudraCT-2019-001878-28	University Hospital Birmingham NHS Foundation Trust	Suspended	Phase I/II	2019	N/A	Yes, in vivo	AAV	N/A	Metabolic (Other)	Phenylketonuria (PKU)
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	NCT04684940 EudraCT-2019-003213-34	Royal Free London 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	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 EudraCT-2011-001953-10	Great Ormond Street Hospital NHS Foundation Trust, London	Completed	Phase II/III	2013	CD34 and/or CD133 stem cells	Yes, ex vivo	Lentivirus	Autologous	Metabolic (Other)	Adrenoleukodystrophy (Adrenomyeloneuropathy/ Schilder-Addison Complex)
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 β0/β0 Genotype, by Transplantation of Autologous CD34+ Stem Cells Transduced Ex Vivo with a Lentiviral βA-T87Q-Globin Vector in Subjects <=50 Years of Age	NCT02906202 EudraCT-2015-004122-33	University College London Hospitals NHS Foundation Trust	In follow-up	Phase III	2017	CD34 and/or CD133 stem cells	Yes, ex vivo	Lentivirus	Autologous	Haematological	β-Thalassemia
bluebird bio Inc	Long-term Follow-up of Subjects with Cerebral Adrenoleukodystrophy who were Treated with Lenti-D Drug Product	NCT02698579 EudraCT-2015-002805-13	Great Ormond Street Hospital NHS Foundation Trust, London	Recruiting	Phase III	2015	CD34 and/or CD133 stem cells	Yes, ex vivo	Lentivirus	Autologous	Metabolic (Other)	Adrenoleukodystrophy
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 βA-T87Q-globin Vector in Subjects <=50 Years of Age	NCT03207009 EudraCT-2016-003611-35	University College London Hospitals NHS Foundation Trust	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 Study of Lenti-D Drug Product After Myeloablative Conditioning Using Busulfan and Fludarabine in Subjects <=17 Years of Age with Cerebral Adrenoleukodystrophy (CALD)	NCT03852498 EudraCT-2018-001145-14	Great Ormond Street Hospital NHS Foundation Trust, London	In follow-up	Phase III	2019	CD34 and/or CD133 stem cells	Yes, ex vivo	Lentivirus	Autologous	Metabolic (Other)	Adrenoleukodystrophy
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 EudraCT-2013-002245-11	University College London Hospitals NHS Foundation Trust	Recruiting	Phase III	2020	CD34 and/or CD133 stem cells	Yes, ex vivo	Lentivirus	Autologous	Haematological	Sickle Cell Disease β-Thalassemia

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Bluebird bio Inc	A Phase III Study Evaluating Gene Therapy by Transplantation of Autologous CD34+ Stem Cells Transduced Ex Vivo with the BB305 Lentiviral Vector in Subjects with Sickle Cell Disease	NCT04293185 EudraCT-2019-000331-63	Two anonymous UK site(s)	Withdrawn	Phase III	2020	CD34 and/or CD133 stem cells	Yes, ex vivo	Lentivirus	Autologous	Haematological	Sickle Cell 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 NHS Foundation Trust, 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)
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 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 SCID
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)	EudraCT-2021-001366-38	Cambridge University Hospitals NHS Foundation Trust/University of Cambridge	In planning/set-up	Phase I/II	2021	Human embryonic stem cells	No	N/A	Allogeneic	Neurological	Parkinson's Disease
Cancer Research UK	A Cancer Research UK Phase I 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 EudraCT-2016-002577-35	University Hospitals Birmingham NHS Foundation Trust University Hospital Southampton NHS Foundation Trust	Recruiting	Phase I	2018	Antigen presenting cells	No	N/A	Allogeneic	Oncology (Other)	Non-Small Cell Lung Cancer
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 EudraCT-2013-004554-17	Great Ormond Street Hospital NHS Foundation Trust, London University College London Hospitals NHS Foundation Trust	Recruiting	Phase I	2016	T cells	Yes, ex vivo	Retrovirus	Autologous	Oncology (Other)	Relapsed or Refractory Neuroblastoma
Cancer Vaccines Ltd	A Phase I/II 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 EudraCT-2012-005426-30	Royal Liverpool University Hospital, Liverpool The Clatterbridge Cancer Centre NHS Foundation Trust	Recruiting	Phase I/II	2017	Antigen presenting cells	No	N/A	Allogeneic	Oncology (Other)	Pancreatic cancer
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

Cell and Gene Therapy Catapult ATMP Clinical Trials Database 2021

Sponsor	Title	Clinical database Numbers	United Kingdom Site(s)	Status	Phase	Year Started	Cell type	Gene modification / gene therapy	Type of Vector Used (if applicable)	Autologous/ Allogeneic	Disease Area	Indication
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 EudraCT-2018-000929-32	UCL Cancer Institute London, University Hospital Southampton NHS Foundation Trust	In follow-up	Phase III	2018	T cells	Yes, ex vivo	Lentivirus	Autologous	Oncology (Haematology)	B-cell Non-Hodgkin Lymphomas
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 EudraCT-2018-001023-38	One unknown site Leeds Teaching Hospitals NHS Trust	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 EudraCT-2017-001465-24	Somers Cancer Research Building, Southampton Christie Hospital NHS Trust, Manchester King's College Hospital NHS Foundation Trust, London UCL Cancer Institute, London Leeds Teaching Hospitals NHS Trust	Recruiting	Phase II/III	2019	T cells	Yes, ex vivo	Lentivirus	Autologous	Oncology (Other)	Oncology
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 EudraCT-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
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 EudraCT-2018-000264-28	King's College Hospital NHS Foundation Trust, London	Recruiting	Phase II	2019	T cells	Yes, ex vivo	Lentivirus	Autologous	Oncology (Other)	Multiple Myeloma
Celgene Corp	A Phase II, Open-label, Single Arm, Multicenter Trial to Evaluate the Safety and Efficacy of JCAR017 (Lisocabtagene Maraleucel) in Adult Subjects with High-risk, Relapsed or Refractory Indolent B-cell Non-Hodgkin Lymphoma (NHL)	NCT04245839 EudraCT-2019-004081-18	The Christie NHS Foundation Trust, Manchester University College London Hospitals NHS Foundation Trust	Recruiting	Phase II	2020	T cells	Yes, ex vivo	Lentivirus	Autologous	Oncology (Haematology)	Non-Hodgkin Lymphoma
Celixir Ltd	A Phase IIB, Randomised, Double-Blinded, Placebo-Controlled Study of the Efficacy and Safety of Intramyocardial Injection of Allogeneic Human Immunomodulatory Progenitor (iMP) cells in Patients Undergoing Coronary Artery Bypass Graft (CABG) Surgery	NCT03515291 EudraCT-2017-000516-42	Unknown UK site(s)	Terminated	Phase II	2018	Other	No	N/A	Allogeneic	Cardiovascular	Ischaemic heart disease post Myocardial Infarction
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 EudraCT-2019-004939-24	Royal Papworth Hospital, London	In planning/set-up	Phase I/II	2020	Mesenchymal stem/stromal cells	No	N/A	Autologous	Respiratory	Bronchopleural Fistula

Cell and Gene Therapy Catapult ATMP Clinical Trials Database 2021

Sponsor	Title	Clinical database Numbers	United Kingdom Site(s)	Status	Phase	Year Started	Cell type	Gene modification / gene therapy	Type of Vector Used (if applicable)	Autologous/ Allogeneic	Disease Area	Indication
CellProthera SAS	Expanded Cell Endocardiac Transplantation (EXCELLENT)	NCT02669810 EudraCT 2014-001476-63	Leeds Teaching Hospitals NHS Trust Newcastle University University of Edinburgh Queen Elizabeth Hospital, Birmingham	Recruiting	Phase I/II	2015	CD34 and/or CD133 stem cells	No	N/A	Autologous	Cardiovascular	Myocardial Infarction Congestive Heart Failure (Heart Failure)
Cook Myosite Inc	A Prospective Nonrandomized Study of Autologous Muscle Derived Cell (AMDC) Transplantation for Treatment of Fecal Incontinence	NCT01600755 EudraCT-2013-004672-35	Royal London Hospital, London	Completed	Phase I/II	2014	Other	No	N/A	Autologous	Gastrointestinal (Other)	Fecal Incontinence
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 EudraCT-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
Cynata Therapeutics Ltd	An Open-label Phasen 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 EudraCT-2016-000070-38	University Hospitals Bristol and Weston NHS Foundation Trust Leeds Teaching Hospitals NHS Trust Liverpool University Hospitals NHS Foundation Trust Manchester University NHS Foundation Trust Nottinghamshire Healthcare NHS Foundation Trust	Completed	Phase I	2017	Mesenchymal stem/stromal cells	No	N/A	Allogeneic	Inflammatory and immune system	Graft Versus Host Disease (GVHD)
Eyevensys 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 EudraCT-2015-001391-22	Bristol Eye Hospital Moorfields Eye Hospital NHS Foundation Trust, London	In follow-up	Phase I/II	2017	N/A	Yes, in vivo	Non-viral	N/A	Ophthalmology	Posterior Uveitis Intermediate Uveitis
Freeline Therapeutics Holdings Plc	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 EudraCT-2017-005080-40	Royal Free London NHS Foundation Trust Oxford University Hospitals NHS Foundation Trust	In follow-up	Phase I/II	2018	N/A	Yes, in vivo	AAV	N/A	Haematological	Haemophilia B
Freeline Therapeutics Holdings Plc	A Phase I/II, Baseline-controlled, Non-randomised, Open-label, Single-ascending Dose Study of a Novel Adeno-associated Viral Vector (FLT190) in Patients with Fabry Disease	NCT04040049 EudraCT-2018-002097-51	Royal Free London NHS Foundation Trust	Recruiting	Phase I/II	2019	N/A	Yes, in vivo	AAV	N/A	Metabolic (Other)	Fabry Disease
Freeline Therapeutics Holdings Plc	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 EudraCT-2019-004645-32	Royal Free London NHS Foundation Trust	Recruiting	Phase II	2020	N/A	Yes, in vivo	AAV	N/A	Metabolic (Other)	Fabry Disease

Cell and Gene Therapy Catapult ATMP Clinical Trials Database 2021

Sponsor	Title	Clinical database Numbers	United Kingdom Site(s)	Status	Phase	Year Started	Cell type	Gene modification / gene therapy	Type of Vector Used (if applicable)	Autologous/ Allogeneic	Disease Area	Indication
Gamida Cell Ltd	A Multicenter, Randomized, Phase III Registration Trial of Transplantation of NiCord®, Ex Vivo Expanded, Umbilical Cord Blood-derived, Stem and Progenitor Cells, versus Unmanipulated Umbilical Cord Blood for Patients with Hematological Malignancies	NCT02730299 EudraCT-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)
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 EudraCT-2012-000242-35	Great Ormond Street Hospital NHS Foundation Trust, London Royal Free London NHS Foundation Trust University College London Hospitals NHS Foundation Trust	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
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 (GTG002-07 and GTG003-08)	NCT02333760 EudraCT-2014-000274-20	UCL Institute of Child Health, London	In follow-up	Phase I/II	2014	CD34 and/or CD133 stem cells	Yes, ex vivo	Lentivirus	Autologous	Inflammatory and immune system	Wiskott-Aldrich Syndrome
Genethon SA	Microdystrophin (GNT0004) Gene Therapy Clinical Trial in Duchenne Muscular Dystrophy. A phase I/II/III study with a dose determination part followed by an efficacy and safety evaluation, quadruple blind placebo-controlled part and then by a long term safety follow up part, in ambulant boys.	EudraCT-2020-002093-27	Unknown UK site(s)	Suspended	Phase I/II/III	2021	N/A	Yes, in vivo	AAV	N/A	Neuromuscular	Duchenne Muscular Dystrophy
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 EudraCT-2017-002204-27	Moorfields Eye Hospital NHS Foundation Trust, London	Recruiting	Phase I/II	2017	N/A	Yes, in vivo	AAV	N/A	Ophthalmology	Retinitis Pigmentosa (Retinitis)
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 EudraCT-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
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 EudraCT-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
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 EudraCT-2019-000415-87	GSK Investigational Site, London The Chrisite NHS Foundation Trust, Manchester	Recruiting	Phase II	2019	T cells	Yes, ex vivo	Lentivirus	Autologous	Oncology (Other)	Synovial Sarcoma
GlaxoSmithKline Plc	Long-Term Follow-Up (LTFU) of Participants Treated With GSK Adoptive Cell Therapies	NCT03391778 EudraCT-2018-004888-31	GSK Investigational Site, London, NW1 2PG GSK Investigational Site, Manchester, M20 4BX GSK Investigational Site, Sutton, SM2 5PT	Recruiting	Phase I	2018	T cells	Yes, ex vivo	Lentivirus	Autologous	Oncology (Other)	Various

Cell and Gene Therapy Catapult ATMP Clinical Trials Database 2021

Sponsor	Title	Clinical database Numbers	United Kingdom Site(s)	Status	Phase	Year Started	Cell type	Gene modification / gene therapy	Type of Vector Used (if applicable)	Autologous/ Allogeneic	Disease Area	Indication
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 (GSK3377794) 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 EudraCT-2018-003949-42	GSK Investigational Site, London, WC1E 6AG 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
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 EudraCT-2018-000673-68	Great Ormond Street Hospital 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)
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 1α Short Form (EFS) Lentiviral Vector Encoding for Human ADA Gene in Subjects With Severe Combined Immunodeficiency (SCID) Due to Adenosine Deaminase Deficiency	NCT03765632 EudraCT-2017-001275-23	Great Ormond Street Hospital NHS Foundation Trust, London	In follow-up	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
Great Ormond Street Hospital for Children NHS Trust	Phase I, Open Label Study of CRISPR-CAR Genome Edited T Cells (PBLTT52CAR19) in Relapsed /Refractory B Cell Acute Lymphoblastic Leukaemia	NCT04557436 EudraCT-2019-003462-40	Great Ormond Street Hospital NHS Foundation Trust, London	Recruiting	Phase I	2020	T cells	Yes, ex vivo	Lentivirus	Allogeneic	Oncology (Haematology)	B-Cell Acute Lymphocytic Leukemia
Gyroscope Therapeutics Ltd	FocuS: 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	NCT03846193 EudraCT-2017-003712-39	University Hospital Southampton NHS Foundation Trust London Vision Clinic Moorfields Eye Hospital NHS Foundation Trust, London Sunderland Eye Infirmary Manchester Eye Hospital Bristol Eye Hospital Oxford University Hospitals NHS Foundation Trust	Recruiting	Phase I/II	2018	N/A	Yes, in vivo	AAV	N/A	Ophthalmology	Age-related Macular Degeneration (AMD)
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 EudraCT-2019-003421-22	Royal Eye Hospital, Manchester	Recruiting	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 EudraCT-2020-002431-30	Sunderland Eye Infirmary, Sunderland	Recruiting	Phase II	2020	N/A	Yes, in vivo	AAV	N/A	Ophthalmology	Age Related Macular Degeneration

Cell and Gene Therapy Catapult ATMP Clinical Trials Database 2021

Sponsor	Title	Clinical database Numbers	United Kingdom Site(s)	Status	Phase	Year Started	Cell type	Gene modification / gene therapy	Type of Vector Used (if applicable)	Autologous/ Allogeneic	Disease Area	Indication
Holostem Terapie Avanzate SRL	Multinational, Multicenter, Prospective, Long-term Safety and Efficacy Follow-up Study After Autologous Cultivated Limbal Stem Cells Transplantation (ACLSCT) for Restoration of Corneal Epithelium in Patients With Limbal Stem Cell Deficiency Due to Ocular Burns	NCT03288844 EudraCT-2015-001344-11	Newcastle upon Tyne Hospitals NHS Foundation Trust Moorfields Eye Hospital NHS Foundation Trust, London University of Nottingham, Queen's Medical Centre	Recruiting	Phase IV	2018	Corneal	No	N/A	Autologous	Ophthalmology	Burns Unspecified Ophthalmological Disorders
Holostem Terapie Avanzate SRL	Multinational, Multicenter, Prospective, Open-label, Uncontrolled Clinical Trial to Assess the Efficacy and Safety of Autologous Cultivated Limbal Stem Cells Transplantation (ACLSCT) for Restoration of Corneal Epithelium in Patients with Limbal Stem Cell Deficiency Due to Ocular Burns (HOLOCORE)	NCT02577861 EudraCT-2014-002845-23	Newcastle upon Tyne Hospitals NHS Foundation Trust Moorfields Eye Hospital NHS Foundation Trust, London University of Nottingham, Queen's Medical Centre	In follow-up	Phase IV	2015	Corneal	No	N/A	Autologous	Ophthalmology	Burns Unspecified Ophthalmological Disorders
Holostem Terapie Avanzate SRL	Long-term safety after Holoclar® implant for restoration of corneal epithelium in patients with limbal stem cell deficiency due to ocular burns: observational study of routine clinical practice	EUPAS10043	Newcastle upon Tyne Hospitals NHS Foundation Trust Moorfields Eye Hospital NHS Foundation Trust, London	Recruiting	Phase IV	2016	Corneal	No	N/A	Autologous	Ophthalmology	Burns Unspecified Ophthalmological Disorders
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	NCT04832607 EudraCT-2018-000853-29	Newcastle University Manchester University NHS Foundation Trust Great Ormond Street Hospital NHS Foundation Trust, London University College London Hospitals NHS Foundation Trust	In planning/set-up	Phase III	2020	T cells	No	N/A	Allogeneic	Infectious Disease	CMV (HHV-5) / EBV (HHV-4) / 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
Innovacell AG	Skeletal Muscle-derived Cell Implantation for the Treatment of Fecal Incontinence: A Phase III, Randomized, Controlled, Double Blind, Two Armed Clinical Study	NCT04976153 EudraCT-2021-001376-42	Anonymous UK site(s)	In planning/set-up	Phase III	2021	Unknown	No	N/A	Autologous	Gastrointestinal (Other)	Fecal Incontinence
Instil Bio UK Ltd	Protocol Title: An Open Label, Multi-centre Phase I/IIa Study of Modified and Unmodified Autologous Tumour Infiltrating Lymphocytes (TIL) in Patients With Platinum-resistant Ovarian Cancer	NCT04389229 EudraCT-2019-000106-30	Queens Elizabeth Hospital, Birmingham The Christie NHS Foundation Trust, Manchester	Withdrawn	Phase I/II	2020	T cells	Yes, ex vivo	Unknown	Autologous	Oncology (Other)	Metastatic Ovarian Cancer
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 EudraCT-2016-000297-38	The Christie NHS Foundation Trust, Manchester King's College Hospital NHS Foundation Trust, London Great Ormond Street Hospital NHS Foundation Trust, London	In follow-up	Phase I	2016	T cells	Yes, ex vivo	Lentivirus	Allogeneic	Oncology (Haematology)	Advanced Lymphoid Malignancies

Cell and Gene Therapy Catapult ATMP Clinical Trials Database 2021

Sponsor	Title	Clinical database Numbers	United Kingdom Site(s)	Status	Phase	Year Started	Cell type	Gene modification / gene therapy	Type of Vector Used (if applicable)	Autologous/ Allogeneic	Disease Area	Indication
Intellia Therapeutics Inc	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 EudraCT-2020-002034-32	Clinical Trial Site, London	Recruiting	Phase I	2020	N/A	Yes, in vivo	Non-viral	N/A	Metabolic (Other)	Hereditary Transthyretin Amyloidosis With Polyneuropathy (ATTRv-PN)
Intellia Therapeutics Inc	Phase I/IIa, Single Dose Study Investigating NTLA-5001 in Subjects with Acute Myeloid Leukemia	NCT05066165 EudraCT- 2021-001231-13	Research Site 7, Manchester Research Site 8, London Research Site 9, London Research Site 10, Leeds	In planning/set-up	Phase I/II	2021	T cells	Yes, ex vivo	Unknown	Allogeneic	Oncology (Haematology)	AML
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 EudraCT-2017-000760-15	The Royal Marsden NHS Foundation Trust, London Addenbrooke's Hospital, Cambridge Sarah Cannon Research Institute, London Beatson West of Scotland Cancer Centre	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 EudraCT-2016-003447-11	Bristol Haematology and Oncology Centre, Bristol Sarah Cannon Research Institute, London University College London Hospitals NHS Foundation Trust NHS Greater Glasgow and Clyde, Glasgow	Recruiting	Phase II	2017	T cells	No	N/A	Autologous	Oncology (Other)	Cervical Carcinomas
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 EudraCT-2018-001608-12	Guy's Hospital, London Bristol Haematology and Oncology Centre, Bristol	Recruiting	Phase II	2019	T cells	No	N/A	Autologous	Oncology (Other)	Metastatic Melanoma
Iovance Biotherapeutics Inc	A Phase II Multicenter Study of Autologous Tumor Infiltrating Lymphocytes (LN-145) in Patients With Metastatic Non-Small-Cell Lung Cancer	NCT04614103 EudraCT-2020-003629-45	Unknown UK site(s)	In planning/set-up	Phase II	2021	T cells	No	N/A	Autologous	Oncology (Other)	Metastatic Non Small Cell Lung Cancer
Ixaka 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	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
Ixaka 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 EudraCT-2016-000240-34	Addenbrooke's Hospital, Cambridge University Hospital of Wales, Cardiff	Terminated	Phase III	2017	Bone marrow mononuclear cells	No	N/A	Autologous	Cardiovascular	Ischemic Rest Pain in Subjects With Critical Limb Ischemia (CLI) and Diabetes Mellitus (DM)

Cell and Gene Therapy Catapult ATMP Clinical Trials Database 2021

Sponsor	Title	Clinical database Numbers	United Kingdom Site(s)	Status	Phase	Year Started	Cell type	Gene modification / gene therapy	Type of Vector Used (if applicable)	Autologous/ Allogeneic	Disease Area	Indication
Janssen Research & Development, LLC	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 EudraCT-2019-003595-38	Queen Elizabeth Hospital, Birmingham Bristol Royal Infirmary University College London Hospitals NHS Foundation Trust King's College Hospital NHS Foundation Trust, London Christie Hospital, Manchester Freeman Hospital, Newcastle upon Tyne Cardiff and Vale University Local Health Board	In follow-up	Phase III	2020	T cells	Yes, ex vivo	Lentivirus	Autologous	Oncology (Haematology)	Multiple Myeloma
Janssen Research & Development, LLC	A Phase III Randomized Study Comparing Bortezomib, Lenalidomide and Dexamethasone (VRd) Followed by Ciltacabtagene Autoleucel, a Chimeric Antigen Receptor T Cell (CAR-T) Therapy Directed Against BCMA Versus Bortezomib, Lenalidomide, and Dexamethasone (VRd) Followed by Lenalidomide and Dexamethasone (Rd) Therapy in Participants With Newly Diagnosed Multiple Myeloma for Whom Hematopoietic Stem Cell Transplant is Not Planned as Initial Therapy	EudraCT-2021-001242-35 NCT04923893	University Hospitals Birmingham NHS Foundation Trust Bristol Royal Infirmary Leeds Teaching Hospitals NHS Trust University College London Hospitals NHS Foundation Trust King's College Hospital NHS Foundation Trust, London Manchester Royal Infirmary The Royal Marsden NHS Foundation Trust, London	In planning/set-up	Phase III	2021	T cells	Yes, ex vivo	Lentivirus	Autologous	Oncology (Haematology)	Multiple Myeloma
Keele University	Autologous Chondrocyte Transplantation/Implantation Versus Existing Treatments	ISRCTN48911177	Cardiff University Keele University University of Birmingham University of Southampton Wessex Institute of Technology	Completed	Phase III	2004	Chondrocytes	No	N/A	Autologous	Musculoskeletal	Musculoskeletal Disorders
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 EudraCT-2016-004672-21	St James University Hospital, Leeds Royal Liverpool University Hospital, Hammersmith Hospital, London Manchester Royal Infirmary	Withdrawn	Phase III	2017	T cells	No	N/A	Allogeneic	Oncology (Haematology)	AML / ALL
King's College London	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 EudraCT-2017-000170-11 ISRCTN97547683	Guy's Hospital, London St Thomas' Hospital, London	Completed	Phase I/II	2016	T cells	No	N/A	Autologous	Gastrointestinal (Other)	Crohn's Disease
King's College London	Phase I Trial: T4 Immunotherapy of Head and Neck Cancer	NCT01818323 EudraCT-2012-001654-25 ISRCTN81726461	Guy's and St Thomas' NHS Foundation Trust, London	Recruiting	Phase I	2015	T cells	Yes, ex vivo	Retrovirus	Autologous	Oncology (Other)	Head And Neck Cancer
Kite Pharma Inc	A Phase III, Randomized, Open-label Study Evaluating Efficacy of Axicabtagene Ciloleucel Versus Standard of Care Therapy in Subjects with Relapsed/Refractory Diffuse Large B Cell Lymphoma	NCT03391466 EudraCT-2017-002261-22	University College London Hospitals NHS Foundation Trust The Christie NHS Foundation Trust, Manchester Barts Health NHS Trust, London University Hospitals Birmingham NHS Foundation Trust The Royal Marsden NHS Foundation Trust, London	In follow-up	Phase III	2018	T cells	Yes, ex vivo	Retrovirus	Autologous	Oncology (Haematology)	B-Cell Lymphoma

Cell and Gene Therapy Catapult ATMP Clinical Trials Database 2021

Sponsor	Title	Clinical database Numbers	United Kingdom Site(s)	Status	Phase	Year Started	Cell type	Gene modification / gene therapy	Type of 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 EudraCT-2017-000789-31	Queen Mary University of London	Recruiting	Phase II	2019	N/A	Yes, in vivo	Adenovirus	N/A	Cardiovascular	Refractory Angina Pectoris
Lysogene SAS	An Open-label Adaptive-design Study of Intracisternal Adenoassociated Viral Vector Serotype rh.10 Carrying the Human β-Galactosidase cDNA for Treatment of GM1 Gangliosidosis	NCT04273269	Manchester University NHS Foundation Trust, Manchester, United Kingdom, M13 9WL	Recruiting	Phase I/II	2021	N/A	Yes, in vivo	AAV	N/A	Neurological	GM1 Gangliosidosis
Lysogene SAS	Open-label, Single-arm, Multi-center Study of Intracerebral Administration of Adeno-associated Viral (AAV) Serotype rh.10 Carrying Human N-sulfoglucosamine Sulfohydrolase (SGSH) cDNA for Treatment of Mucopolysaccharidosis Type IIIA	NCT03612869 EudraCT-2018-000195-15	Great Ormond Street Hospital NHS Foundation Trust, London	In follow-up	Phase II/III	2019	N/A	Yes, in vivo	AAV	N/A	Metabolic (Other)	Mucopolysaccharidosis IIIA (MPS IIA)
Mario Negri Institute for Pharmacological Research	Novel Stromal Cell Therapy for Diabetic Kidney Disease (NEPHSTROM Study)	NCT02585622 EudraCT -2016-000661-23	Belfast Health and Social Care Trust - Belfast City HospitaL University Hospital 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 UK II Ltd	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 EudraCT-2016-003967-21	Moorfields Eye Hospital NHS Foundation Trust London Leeds Teaching Hospitals NHS Trust	Completed	Phase I/II	2017	N/A	Yes, in vivo	AAV	N/A	Ophthalmology	X-Linked Retinitis Pigmentosa
MeiraGTx UK II Ltd	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-OPTIRPE65) for Gene Therapy of Adults and Children with Retinal Dystrophy Owing to Defects in RPE65 (LCA2)	NCT02946879 EudraCT-2016-000898-20	Moorfields Eye Hospital NHS Foundation Trust, London	In follow-up	Phase I/II	2016	N/A	Yes, in vivo	AAV	N/A	Ophthalmology	Leber Congenital Amaurosis (LCA)
MeiraGTx UK II Ltd	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 EudraCT-2016-003856-59	Moorfields Eye Hospital NHS Foundation Trust, London	In follow-up	Phase I/II	2017	N/A	Yes, in vivo	AAV	N/A	Ophthalmology	Achromatopsia
MeiraGTx UK II Ltd	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 and Adults with Achromatopsia Owing to Defects in CNGA3	NCT03758404 EudraCT-2018-003431-29	Moorfields Eye Hospital NHS Foundation Trust, London	Completed	Phase I/II	2019	N/A	Yes, in vivo	AAV	N/A	Ophthalmology	Achromatopsia

Cell and Gene Therapy Catapult ATMP Clinical Trials Database 2021

Sponsor	Title	Clinical database Numbers	United Kingdom Site(s)	Status	Phase	Year Started	Cell type	Gene modification / gene therapy	Type of Vector Used (if applicable)	Autologous/ Allogeneic	Disease Area	Indication
MeiraGTx UK II Ltd	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 EudraCT-2018-000425-31	Moorfields Eye Hospital NHS Foundation Trust London Leeds Teaching Hospitals NHS Trust	In follow-up	Phase I/II	2018	N/A	Yes, in vivo	AAV	N/A	Ophthalmology	X-Linked Retinitis Pigmentosa
MeiraGTx UK II Ltd	Follow-up Phase III Randomized, Controlled Study of AAV5-RPGR for the Treatment of X-linked Retinitis Pigmentosa Associated with Variants in the RPGR Gene	NCT04794101 EudraCT-2020-002255-37	Anonymous UK site(s)	Recruiting	Phase III	2021	N/A	Yes, in vivo	AAV	N/A	Ophthalmology	X-Linked Retinitis Pigmentosa
MeiraGTx UK II Ltd	Phase III Randomized, Controlled Study of AAV5-RPGR for the Treatment of X-linked Retinitis Pigmentosa Associated with Variants in the RPGR Gene	NCT04671433 EudraCT-2020-002873-88	Anonymous UK site(s)	Recruiting	Phase III	2021	N/A	Yes, in vivo	AAV	N/A	Ophthalmology	X-Linked Retinitis Pigmentosa
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 EudraCT-2010-020497-41	The Newcastle upon Tyne Hospitals NHS Foundation Trust One unknown site	In follow-up	Phase II	2013	Mesenchymal stem/stromal cells	No	N/A	Allogeneic	Cardiovascular	Myocardial Infarction
MiNA Therapeutics Ltd	A First-in-Human, Multi-centre, Open-label, Phase 1a/b Clinical Study With RNA Oligonucleotide Drug MTL-CEBPA to Investigate Its Safety, Tolerability, and Antitumour Activity in Patients With Advanced Liver Cancer	NCT02716012	University Hospitals Birmingham NHS Foundation Trust Cambridge University Hospitals NHS Trust Beatson West of Scotland Cancer Centre The Clatterbridge Cancer Centre NHS Foundation Trust Guy's and St. Thomas' NHS Foundation Trust, London Imperial College Healthcare NHS Trust, London University College London Hospitals NHS Foundation Trust 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
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 106 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 EudraCT-2015-002125-19	Cambridge University Hospitals NHS Foundation Trust	Recruiting	Phase II	2017	Adipose-derived stem cells	No	N/A	Autologous	Musculoskeletal	Osteoarthritis (knee)
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 EudraCT-2011-005493-37	Moorfields Eye Hospital NHS Foundation Trust, London	In planning/set-up	Phase I	2021	Retinal	No	N/A	Allogeneic	Ophthalmology	Acute Wet Age Related Macular Degeneration
Neurotech Pharmaceuticals Inc	A Phase III Multicenter Randomized, Sham Controlled, Study to Determine the Safety and Efficacy of NT-501 in Macular Telangiectasia Type 2	NCT03316300 EudraCT-2017-003234-82	Oxford Eye Hospital Moorfields Eye Hospital NHS Foundation Trust, London	In follow-up	Phase III	2018	Retinal	Yes, ex vivo	Plasmid	Allogeneic	Ophthalmology	Macular Telangectasia

Cell and Gene Therapy Catapult ATMP Clinical Trials Database 2021

Sponsor	Title	Clinical database Numbers	United Kingdom Site(s)	Status	Phase	Year Started	Cell type	Gene modification / gene therapy	Type of Vector Used (if applicable)	Autologous/ Allogeneic	Disease Area	Indication
NHS Lothian University of Edinburgh Academic and Clinical Central Office for Research and Development	Macrophage Therapy for Liver Cirrhosis (MATCH)	EudraCT-2015-000963-15 ISRCTN10368050	Royal Infirmary of Edinburgh NHS Lothian North Bristol NHS Trust University Hospitals Bristol and Weston NHS Foundation Trust University of Bristol University of Edinburgh Western General Hospital	In follow-up	Phase I/II	2016	Other	No	N/A	Autologous	Gastrointestinal (Liver)	Liver cirrhosis
Novartis AG	Long Term Follow-Up of Patients Exposed to Lentiviral-Based CAR T-Cell Therapy	NCT02445222 EudraCT-2014-001673-14	Unknown UK site(s)	Recruiting	N/A	2020	T cells	Yes, ex vivo	Lentivirus	Autologous	Oncology (Haematology)	Unspecified B-Cell Lymphomas B-Cell Leukemia
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 EudraCT-2017-004385-94	Queen Elizabeth Hospital NHS Foundation Trust	In follow-up	Phase II	2018	T cells	Yes, ex vivo	Lentivirus	Autologous	Oncology (Haematology)	Follicular Lymphoma
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 EudraCT-2016-002966-29	Queen Elizabeth Hospital, Birmingham University College London Hospitals NHS Foundation Trust	In follow-up	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)	NCT03610724 EudraCT-2017-005019-15	2 Unknown UK sites Royal Manchester Children's Hospital	In follow-up	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 EudraCT-2017-002116-14	Novartis Investigative Site, London, WC1N 3JH Novartis Investigative Site, London WC1E 6HX	Recruiting	Phase II	2019	T cells	Yes, ex vivo	Lentivirus	Autologous	Oncology (Haematology)	B-cell Acute Lymphoblastic Leukaemia
Novartis AG	A Phase IIIb, Open-label, Single-arm, Single-dose, Multicenter Study to Evaluate the Safety, Tolerability and Efficacy of Gene Replacement Therapy With Intravenous OAV101 (AVXS-101) in Pediatric Patients with Spinal Muscular Atrophy (SMA)	NCT04851873 EudraCT-2020-005995-37	Great North Children's Hospital, Newcastle	Recruiting	Phase III	2021	N/A	Yes, in vivo	AAV	N/A	Neuromuscular	Spinal Muscular Atrophy (SMA)
Novartis Gene Therapies	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 EudraCT-2017-000266-29	Great Ormond Street Hospital NHS Foundation Trust, London The John Walton Muscular Dystrophy Research Centre, Newcastle The Great North Children's Hospital, Newcastle upon Tyne	Completed	Phase III	2018	N/A	Yes, in vivo	AAV	N/A	Neuromuscular	Spinal Muscular Atrophy

Cell and Gene Therapy Catapult ATMP Clinical Trials Database 2021

Sponsor	Title	Clinical database Numbers	United Kingdom Site(s)	Status	Phase	Year Started	Cell type	Gene modification / gene therapy	Type of Vector Used (if applicable)	Autologous/ Allogeneic	Disease Area	Indication
Novartis Gene Therapies	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 EudraCT-2017-004087-35	Great Ormond Street Hospital NHS Foundation Trust, London	Completed	Phase III	2018	N/A	Yes, in vivo	AAV	N/A	Neuromuscular	Spinal Muscular Atrophy
Novartis Gene Therapies	A Long-term Follow-up Study of Patients in the Clinical Trials for Spinal Muscular Atrophy Receiving AVXS-101	NCT04042025 EudraCT-2019-002611-26	Great Ormond Street Hospital NHS Foundation Trust, London The Newcastle Upon Tyne Hospitals NHS Foundation Trust	Recruiting	Phase IV	2020	N/A	Yes, in vivo	AAV	N/A	Neuromuscular	Spinal Muscular Atrophy (SMA)
Orbsen	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	EudraCT-2019-003404-13	University Hospitals Birmingham NHS Foundation Trust	In planning/set-up	Phase II	2020	Mesenchymal stem/stromal cells	No	N/A	Allogeneic	Gastrointestinal (Liver)	Lupus nephritis, PSC, Rheumatoid arthitis, crohns disease
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	Great Ormond Street Hospital NHS Foundation Trust, 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
Oxford BioMedica	A Multicenter, Open-label Study to Determine the Long Term Safety, Tolerability and Efficacy of Prosavin in Patients with Bilateral, Idiopathic Parkinson's Disease	NCT01856439 EudraCT-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
Passage Bio Inc	A Phase I/II Open-label, Multicenter Dose-ranging and Confirmatory Study to Assess the Safety, Tolerability and Efficacy of PBKR03Administered to Pediatric Subjects with Early Infantile Krabbe Disease (Globoid Cell Leukodystrophy)	NCT04771416 EudraCT-2020-005229-95	Manchester University	In planning/set-up	Phase I/II	2021	N/A	Yes, in vivo	AAV	N/A	Neurological	Krabbe Disease
Passage Bio Inc	A Phase I/II Open-label, Multicenter Study to Assess the Safety, Tolerability and Efficacy of a Single Dose of PBGM01 Delivered Into the Cisterna Magna of Subjects with Type 1 (Early Onset) and Type IIa (Late Onset) Infantile GM1 Gangliosidosis	NCT04713475 EudraCT-2020-001109-22	Great Ormond Street Hospital NHS Foundation Trust, London	In planning/set-up	Phase I/II	2021	N/A	Yes, in vivo	AAV	N/A	Genetic Disorders	GM1 Gangliosidosis
Passage Bio Inc	A Phase 1b Open-Label, Multicenter, Dose-Escalation Study to Assess the Safety, Tolerability, and Pharmacodynamic Effects of a Single Dose of PBFT02 Delivered Into the Cisterna Magna of Adult Subjects With Frontotemporal Dementia and Mutations in the Progranulin Gene	NCT04747431 EudraCT-2020-004499-17	University of Cambridge	In planning/set-up	Phase I	2021	N/A	Yes, in vivo	AAV	N/A	Neurological	Frontotemporal Dementia
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 EudraCT-2018-003086-33	Newcastle upon Tyne Hospitals NHS FT, Newcastle Upon Tyne Glasgow Royal Infirmary	Recruiting	Phase III	2020	N/A	Yes, in vivo	AAV	N/A	Haematological	Hemophilia B (Factor IX Deficiency)

Cell and Gene Therapy Catapult ATMP Clinical Trials Database 2021

Sponsor	Title	Clinical database Numbers	United Kingdom Site(s)	Status	Phase	Year Started	Cell type	Gene modification / gene therapy	Type of Vector Used (if applicable)	Autologous/ Allogeneic	Disease Area	Indication
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 EudraCT-2019-002921-31	3 Unknown UK sites	Recruiting	Phase III	2020	N/A	Yes, in vivo	AAV	N/A	Neuromuscular	Duchenne Muscular Dystrophy
Pfizer Inc	Phase 3, 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%)	NCT04370054 EudraCT-2019-004451-37	Guy's and St. Thomas' NHS Foundation Trust London	In follow-up	Phase III	2020	N/A	Yes, in vivo	AAV	N/A	Haematological	Haemophilia A
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 Unsuitable for Revascularization	NCT03006770 EudraCT-2015-005532-18	Hull And East Yorkshire Hospitals NHS Trust St George's Hospital, London King's College Hospital NHS Foundation Trust, London Southmead Hospital, Bristol	Terminated	Phase III	2017	Mesenchymal stem/stromal cells	No	N/A	Allogeneic	Cardiovascular	Critical Limb Ischemia (CLI)
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 EudraCT-2017-005165-49	Norfolk and Norwich University Hospital John Radcliffe Hospital, Oxford	In follow-up	Phase III	2019	Mesenchymal stem/stromal cells	No	N/A	Allogeneic	Musculoskeletal	Bone Fracture Muscle Injury
Prevail Therapeutics Inc	An Open-label, Phase I/II Study to Evaluate the Safety and Efficacy of Single-dose PR001a in Infants with Type II Gaucher Disease	NCT04411654	Manchester Centre for Genomic Medicine, 6th Floor, St Mary's Hospital	Recruiting	Phase I/II	2021	N/A	Yes, in vivo	AAV	N/A	Metabolic (Other)	Type 2 Gaucher disease
Prevail Therapeutics Inc	A Phase I/II Ascending Dose Study to Evaluate the Safety and Effects on Progranulin Levels of PR006A in Patients with Fronto-temporal Dementia with Progranulin Mutations (FTD-GRN)	NCT04408625 EudraCT-2019-003159-12	Unknown UK site(s)	In planning/set-up	Phase I/II	2021	N/A	Yes, in vivo	AAV	N/A	Neurological	Frontotemporal Dementia
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) - DHELIVER	NCT04229901 EudraCT-2019-003051-11	Unknown UK site(s)	Recruiting	Phase II	2020	Other	No	N/A	Allogeneic	Gastrointestinal (Liver)	Acute on Chronic Liver Failure (ACLF)
PsiOxus Therapeutics Ltd	A Multicentre, Open-label, Dose-escalating, Phase Ib, Study of Intravenous Dosing of NG-641, as Monotherapy or in Combination with Pembrolizumab in Patients with Surgically Resectable Squamous Cell Carcinoma of the Head and Neck	NCT04830592	Cardiff & Vale University LHB, Cardiff The Clatterbridge Cancer Centre NHS Foundation Trust The Royal Marsden NHS Foundation Trust, London	Recruiting	Phase I	2021	N/A	Yes, in vivo	Oncolytic Virus	N/A	Oncology (Other)	Squamous Cell Carcinoma of the Head and Neck
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

Cell and Gene Therapy Catapult ATMP Clinical Trials Database 2021

Sponsor	Title	Clinical database Numbers	United Kingdom Site(s)	Status	Phase	Year Started	Cell type	Gene modification / gene therapy	Type of Vector Used (if applicable)	Autologous/ Allogeneic	Disease Area	Indication
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 EudraCT-2019-004547-77	Oxford Eye Hospital	Recruiting	Phase I/II	2020	Retinal	No	N/A	Allogeneic	Ophthalmology	Retinitis Pigmentosa (Retinitis)
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 EudraCT-2016-004548-12	The Clatterbridge Cancer Centre NHS Foundation Trust Oxford University Hospitals NHS Foundation Trust Leeds Teaching Hospitals NHS Trust The Royal Marsden NHS Foundation Trust, London University of Leeds Beatson West of Scotland Cancer Centre	Recruiting	Phase I/II	2017	N/A	Yes, in vivo	Oncolytic Virus	N/A	Oncology (Other)	Melanoma
Replimune Ltd	A Randomized, Controlled, Open-label, Phase II Study of Cemiplimab as a Single Agent and in Combination with RP1 in Patients with Advanced Cutaneous Squamous Cell Carcinoma	NCT04050436 EudraCT-2018-003964-30	Unknown UK site(s)	In planning/set-up	Phase II	2019	N/A	Yes, in vivo	Oncolytic Virus	N/A	Oncology (Other)	Cutaneous Squamous Cell Carcinoma
Replimune Ltd	An Open-Label, Multicenter, Phase I/II Study of RP2 as a Single Agent and in Combination With PD1 Blockade in Patients With Solid Tumors	NCT04336241	The Clatterbridge Cancer Centre NHS Foundation Trust The Royal Marsden NHS Foundation Trust, London Churchill Hospital, Oxford	Recruiting	Phase I/II	2019	N/A	Yes, in vivo	Oncolytic Virus	N/A	Oncology (Other)	Advanced solid tumors
Replimune Ltd	An Open-label, Multicenter, Phase 1 Study of RP3 as a Single Agent and in Combination with PD-1 Blockade in Patients with Solid Tumors	NCT04735978	The Clatterbridge Cancer Centre NHS Foundation Trust The Royal Marsden NHS Foundation Trust, London Churchill Hospital, Oxford	Recruiting	Phase I	2020	N/A	Yes, in vivo	Oncolytic Virus	N/A	Oncology (Other)	Advanced Solid Tumor
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 EudraCT-2018-001009-98	Great Ormond Street Hospital NHS Foundation Trust, London King's College London; St John's Institute of Dermatology, London	In follow-up	Phase I/II	2018	Mesenchymal stem/stromal cells	No	N/A	Allogeneic	Dermatological	Epidermolysis Bullosa (EB)
Rocket Pharmaceuticals Inc	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 EudraCT-2020-000517-33	Great Ormond Street Hospital NHS Foundation Trust, London	In follow-up	Phase I/II	2020	CD34 and/or CD133 stem cells	Yes, ex vivo	Lentivirus	Autologous	Inflammatory and immune system	Leukocyte Adhesion Deficiency-I (LAD-I)
Rocket Pharmaceuticals Inc	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 EudraCT-2018-002502-31	Great Ormond Street Hospital NHS Foundation Trust, London	Recruiting	Phase II	2020	CD34 and/or CD133 stem cells	Yes, ex vivo	Lentivirus	Autologous	Haematological	Fanconi Anemia Subtype A
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 EudraCT-2018-000206-28	Unknown UK site(s)	Completed	Phase I/II	2018	N/A	Yes, in vivo	AAV	N/A	Metabolic (Other)	Mucopolysaccharidosis type I (MPS I)
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 EudraCT-2018-000192-33	3 Unknown sites Royal Manchester Children's Hosptial	Terminated	Phase I/II	2018	N/A	Yes, in vivo	AAV	N/A	Metabolic (Other)	Mucopolysaccharidosis II (MPS II)

Cell and Gene Therapy Catapult ATMP Clinical Trials Database 2021

Sponsor	Title	Clinical database Numbers	United Kingdom Site(s)	Status	Phase	Year Started	Cell type	Gene modification / gene therapy	Type of Vector Used (if applicable)	Autologous/ Allogeneic	Disease Area	Indication
Sangamo Therapeutics Inc	A Phase I/II, Multicenter, Open-label, Single-dose, Dose-ranging Study to Assess the Safety and Tolerability of ST-920, an AAV2/6 Human Alpha Galactosidase a Gene Therapy, in Subjects with Fabry Disease	NCT04046224 EudraCT-2019-000667-24	Queen Elizabeth NHS Foundation Trust Cambridge University Hospitals NHS Foundation Trust Royal Free London NHS Foundation Trust	Recruiting	Phase I/II	2019	N/A	Yes, in vivo	AAV	N/A	Metabolic (Other)	Fabry Disease
Sangamo Therapeutics Inc	Multicentre Open-label Single Ascending Dose Dose-ranging Phase I/IIa Study to Evaluate Safety and Tolerability of an Autologous Antigen-Specific Chimeric Antigen Receptor TRegulatory Cell Therapy in Living Donor Renal Transplant Recipients	NCT04817774 EudraCT-2019-001730-34	Oxford University Hospitals NHS Foundation Trust	Recruiting	Phase I/II	2021	T cells	Yes, ex vivo	Lentivirus	Autologous	Inflammatory and immune system	Kidney Transplant Rejection
Scancell Holdings Plc	A Phase II, Multicenter, Open-label Study of SCIB1 in Patients with Advanced Unresectable Melanoma Receiving Pembrolizumab	NCT04079166 EudraCT-2018-002844-10	Velindre University NHS Trust, Cardiff East and North Hertfordshire NHS Trust Nottingham University Hospitals NHS Trust Oxford University Hospitals NHS Foundation Trust	Recruiting	Phase II	2019	N/A	Yes, in vivo	Non-viral	N/A	Oncology (Other)	Advanced unresectable melanoma
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 EudraCT-2016-000296-24	The Christie NHS Foundation Trust, Manchester King's College Hospital NHS Foundation Trust, London	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 EudraCT-2015-004293-15	Great Ormond Street Hospital NHS Foundation Trust, London	Withdrawn	Phase I	2016	T cells	Yes, ex vivo	Lentivirus	Allogeneic	Oncology (Haematology)	Relapsed/Refractory B Acute Lymphoblastic Leukemia
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 EudraCT-2019-004210-33	Clinical Study Site, London Clinical Study Site, Southampton Manchester Royal Infirmary	Suspended	Phase I/II	2020	Retinal	Yes, ex vivo	Non-viral	Allogeneic	Haematological	Hemophilia A (Factor VIII Deficiency)
Sio Gene Therapies Inc	A Phase I/II Safety and Dose Evaluation Study of OXB-102 (AXO-Lenti-PD) in Patients With Bilateral Idiopathic Parkinson's Disease (SUNRISE-PD)	NCT03720418	University of Cambridge, Centre for Brain Repair The National Hospital for Neurology and Neurosurgery, London University College London Hospitals NHS Foundation Trust National Institute for Health Research, Southampton	In follow-up	Phase I/II	2018	N/A	Yes, in vivo	Lentivirus	N/A	Neurological	Parkinson's Disease
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 EudraCT-2012-002814-38	The Clatterbridge Cancer Centre NHS Foundation Trust University Hospitals Birmingham NHS Foundation Trust Bristol Haematology and Oncology Centre Cambridge University Hospitals NHS Foundation Trust University of Surrey, Guildford Royal Free London NHS Foundation Trust 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

Cell and Gene Therapy Catapult ATMP Clinical Trials Database 2021

Sponsor	Title	Clinical database Numbers	United Kingdom Site(s)	Status	Phase	Year Started	Cell type	Gene modification / gene therapy	Type of Vector Used (if applicable)	Autologous/ Allogeneic	Disease Area	Indication
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 EudraCT-2019-001283-30	Salford Royal MHS Foundation Trust, Salford, England	Recruiting	Phase I/II	2020	N/A	Yes, in vivo	AAV	N/A	Metabolic (Other)	Lysosomal Storage Disorder
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 EudraCT-2005-005711-17	Royal Free London NHS Foundation Trust	Suspended	Phase I/II	2009	N/A	Yes, in vivo	AAV	N/A	Haematological	Hemophilia B (Factor IX 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)	EUPAS24267	St Mark's Hospital, Northem General Hospital	Recruiting	Phase IV	2018	Mesenchymal stem/stromal cells	No	N/A	Allogeneic	Gastrointestinal (Other)	Crohn's Disease and Complex Perianal Fistulas
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	EudraCT-2010-022072-31 ISRCTN98997175	The Robert Jones and Agnes Hunt Orthopaedic Hospital NHS Foundation Trust	Recruiting	Phase II	2013	Chondrocytes	No	N/A	Autologous	Musculoskeletal	Knee Osteoarthritis
The Royal Marsden NHS Foundation Trust	A Phase I/II Study of the Safety and Efficacy of Talimogene Laherparepvec (T-VEC) Delivered by Intra-tumoural Injection in Combination with Isolated Limb Perfusion with Melphalan and Tumour Necrosis Factor-α in Patients with Advanced Extremity Tumours Including Metastatic Melanoma	NCT03555032	The Royal Marsden NHS Foundation Trust, London	Completed	Phase I/II	2018	N/A	Yes, in vivo	Oncolytic Virus	N/A	Oncology (Other)	Melanoma
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.	EudraCT-2019-001825-28	Royal Manchester Children's Hospital	In planning/set-up	Phase I/II	2019	Other	Yes, ex vivo	Lentivirus	Autologous	Neuromuscular	Duchenne Muscular Dystrophy
The University of Manchester	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 EudraCT-2019-002051-42	Manchester University Hospital NHS Foundation Trust	In follow-up	Phase I/II	2020	CD34 and/or CD133 stem cells	Yes, ex vivo	Lentivirus	Autologous	Metabolic (Other)	Mucopolysaccharidosis IIIA (MPS IIIA)
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 EudraCT-2017-000725-12	Glasgow Royal Infirmary (GRI), 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 Northern General Hospital, Sheffield	Recruiting	Phase III	2019	Adipose-derived stem cells	No	N/A	Allogeneic	Gastrointestinal (Other)	Crohn's Disease

Cell and Gene Therapy Catapult ATMP Clinical Trials Database 2021

Sponsor	Title	Clinical database Numbers	United Kingdom Site(s)	Status	Phase	Year Started	Cell type	Gene modification / gene therapy	Type of Vector Used (if applicable)	Autologous/ Allogeneic	Disease Area	Indication
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 EudraCT-2011-005798-22	Royal Devon and Exeter Hospital, Exeter University Hospital of Coventry and Warwickshire, Coventry (Withdrawn)	In follow-up	Phase III	2013	Chondrocytes	No	N/A	Autologous	Musculoskeletal	Traumatic Articular Cartilage Defects of the Knee
Transgene SA	A Dose-escalation and Phase IIa Study of TG6002 Plus Flucytosine in Patients With Unresectable Colorectal Cancer With Liver Metastases	NCT04194034 EudraCT-2018-004103-39	St James's University Hospital, Leeds	Recruiting	Phase II	2020	N/A	Yes, in vivo	Oncolytic Virus	N/A	Oncology (Other)	Colorectal Neoplasms
Transgene SA	A Randomized Phase I Trial in Patients with Newly Diagnosed, Locoregionally Advanced Squamous Cell Carcinoma of the Head and Neck (SCCHN) Evaluating a Mutanome-Directed Immunotherapy	NCT04183166 EudraCT-2018-003267-58	The Clatterbridge Cancer Centre NHS Foundation Trust Aintree University Hospital NHS Fondation Trust, Liverpool University Hospital Southampton NHS Foundation Trust	Recruiting	Phase I	2019	N/A	Yes, in vivo	MVA	N/A	Oncology (Other)	Squamous cell carcinoma of the head and neck
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 EudraCT-2017-003169-82	Beatson West of Scotland Cancer Centre Derriford Hospital, Plymouth The Royal Marsden NHS Foundation Trust, London Guy's and St. Thomas' NHS Trust, London Wythenshawe Hospital UHSM, Manchester Churchill Hospital, Oxford	In follow-up	Phase III	2019	N/A	Yes, in vivo	Adenovirus	N/A	Oncology (Other)	Malignant Pleural Mesothelioma
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	NCT04301011 2021-001001-79	Unknown UK site(s)	In planning/set-up	Phase I/II	2021	N/A	Yes, in vivo	Oncolytic Virus	N/A	Oncology (Other)	Solid Tumors
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 EudraCT-2016-001057-40	Queen Elizabeth Hospital, Birmingham National Hospital for Neurology and Neurosurgery, London	Completed	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 EudraCT-2016-003430-25	Haemostasis & Thrombosis Centre, Basingstoke Manchester Haemophilia Comprehensive Care Center, Manchester	Completed	Phase I/II	2017	N/A	Yes, in vivo	AAV	N/A	Haematological	Hemophilia B (Factor IX Deficiency)
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 EudraCT-2018-000156-18	Queen Elizabeth Hospital, Birmingham	Recruiting	Phase I/II	2018	N/A	Yes, in vivo	AAV	N/A	Metabolic (Other)	Ornithine-Transcarbamylase Deficiency

Cell and Gene Therapy Catapult ATMP Clinical Trials Database 2021

Sponsor	Title	Clinical database Numbers	United Kingdom Site(s)	Status	Phase	Year Started	Cell type	Gene modification / gene therapy	Type of Vector Used (if applicable)	Autologous/ Allogeneic	Disease Area	Indication
UniQure Biopharma B.V	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 EudraCT-2017-004305-40	University Hospitals Bristol and Weston NHS Foundation Trust Addenbrooke's Hospital, Cambridge, The Royal London Hospital, London University Hospital Southampton NHS Foundation Trust	In follow-up	Phase III	2018	N/A	Yes, in vivo	AAV	N/A	Haematological	Haemophilia B
UniQure Biopharma B.V	A Phase Ib/II Study to Explore Safety, Tolerability, and Efficacy Signals of Multiple Ascending Doses of Striatally-administered rAAV5-miHTT Total Huntingtin Gene (HTT) Lowering Therapy (AMT 130) in Early Manifest Huntington Disease	EudraCT-2020-001461-36	Cardiff University, University College London Hospitals NHS Foundation Trust	Recruiting	Phase I/II	2021	N/A	Yes, in vivo	AAV	N/A	Neurological	Huntington Disease
University College London	Adoptive Immunotherapy with CD25/71 Allodepleted Donor T Cells to Improve Immunity After Unrelated Donor Stem Cell Transplant	NCT01827579 EudraCT-2013-000872-14	Manchester Royal Infirmary University College London Hospitals NHS Foundation Trust	Completed	Phase I/II	2013	T cells	No	N/A	Allogeneic	Oncology (Haematology)	Haematological Malignancies
University College London	COBALT: Evaluation of CAR19 T-cells as an Optimal Bridge to Allogeneic Transplantation	NCT02431988 Eudra CT-2015-000348-40	University College London Hospitals NHS Foundation Trust	In follow-up	Phase I	2016	T cells	Yes, ex vivo	Lentivirus	Autologous	Oncology (Other)	Diffuse Large B-Cell Lymphoma
University College London	Immunotherapy with CD19 CAR Redirected T-cells for High Risk, Relapsed Pediatric CD19+ Acute Lymphoblastic Leukaemia and Other Hematological Malignancies	NCT02443831 EudraCT-2015-001144-10	Royal Manchester Children's Hospital, Manchester Great Ormond Street Hospital NHS Foundation Trust, London University College London Hospitals NHS Foundation Trust Manchester Royal Infirmary	In follow-up	Phase I	2016	T cells	Yes, ex vivo	Lentivirus	Autologous	Oncology (Haematology)	Haematological malignancies
University College London	Chimeric Antigen Receptor (CAR)19 Donor Lymphocytes for Relapsed Cluster of Differentiation (CD)19+ Malignancies Following Allogeneic Transplantation (CARD)	NCT02893189 EudraCT-2016-001869-85	University College London Hospitals NHS Foundation Trust	In follow-up	Phase I	2017	T cells	Yes, ex vivo	Unknown	Autologous	Oncology (Other)	CD19+ Malignancies
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 EudraCT-2016-004027-22	University College London Hospitals NHS Foundation Trust The Christie NHS Foundation Trust, Manchester Beatson West of Scotland Cancer Centre / Queen Elizabeth University Hospital	Recruiting	Phase I/II	2017	T cells	Yes, ex vivo	Lentivirus	Autologous	Oncology (Haematology)	CD19+ leukaemia
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 EudraCT-2016-000925-38	Royal Free London NHS Foundation Trust	Recruiting	Phase I/II	2017	N/A	Yes, in vivo	AAV	N/A	Haematological	Hemophilia A (Factor VIII Deficiency)
University College London	Immunotherapy with Tacrolimus Resistant EBV CTL for Lymphoproliferative Disease after Solid Organ Transplant	NCT03131934 EudraCT 2017-001020-22	Great Ormond Street Hospital NHS Foundation Trust, London King's College Hospital, London	Recruiting	Phase I	2019	T cells	Yes, ex vivo	Retrovirus	Autologous	Oncology (Haematology)	Post-Transplant Lymphoproliferative Disorder
University College London	Targeted Stem Cells Expressing TRAIL as a Therapy for Lung Cancer	NCT03298763	University College London Hospitals NHS Foundation Trust	Recruiting	Phase I/II	2019	Mesenchymal stem / stromal cells	Yes, ex vivo	Lentivirus	Allogeneic	Oncology (Other)	Metastatic non-Small Cell Lung Cancer

Cell and Gene Therapy Catapult ATMP Clinical Trials Database 2021

Sponsor	Title	Clinical database Numbers	United Kingdom Site(s)	Status	Phase	Year Started	Cell type	Gene modification / gene therapy	Type of Vector Used (if applicable)	Autologous/ Allogeneic	Disease Area	Indication
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 EudraCT-2017-000852-24	Basingstoke Haemostasis and Thrombosis Centre, Basingstoke Guy's and St Thomas's NHS Foundation Trust, London Newcastle Hospitals NHS Trust, Newcastle Royal Free London NHS Foundation Trust Oxford University Hospitals NHS Foundation Trust University Hospital Southampton NHS Foundation Trust East Kent Hospitals University, Canterbury Oxford University Hospital University of Sheffield, Sheffield Manchester Royal Infirmary	Terminated	Phase I/II	2017	N/A	Yes, in vivo	AAV	N/A	Haematological	Hemophilia B (Factor IX Deficiency)
University College London	Phase I Study of Transfer of Effector Memory T Cells (Tem) Following Allogeneic Stem Cell Transplantation	NCT03836690	University College London Hospitals NHS Foundation Trust	Recruiting	Phase I	2019	T cells	No	N/A	Allogeneic	Oncology (Haematology)	Leukaemia / Lymphoma
University College London	Immunotherapy Using CAR T-cells to Target CD19 for Relapsed/Refractory CD19+ Primary Central Nervous System (CNS) Lymphoma	NCT04443829	University College London Hospitals NHS Foundation Trust	Recruiting	Phase I	2021	T cells	Yes, ex vivo	Unknown	Autologous	Oncology (Other)	Primary CNS Lymphoma
University College London	An Open Label, Phase 1 Study Evaluating the Activity of Modular CAR T for myeloma	NCT04795882 2020-005161-13	Unknown UK site(s)	In planning/set-up	Phase I	2021	T cells	Yes, ex vivo	Lentivirus	Unknown	Oncology (Haematology)	Multiple Myeloma
University of Birmingham	AdUP: A Phase I Clinical Trial of a Replication Defective Type 5 Adenovirus Vector Expressing Nitroreductase and GMCSF (AdNRGM) Given Via Trans-perineal, Template-guided, Intra-prostatic Injection, Followed by Intravenous CB1954, in Patients with Locally Relapsed Hormone-refractory Prostate Cancer	NCT04374240 ISRCTN06254734	Queen Elizabeth Hospital, Birmingham University of Warwick	Completed	Phase I	2013	N/A	Yes, in vivo	Adenovirus	N/A	Oncology (other)	Prostate Cancer
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 EudraCT-2016-000181-36 ISRCTN73586959	Queen Elizabeth Hospital, Birmingham Nottingham University Hospital, Nottingham John Radcliffe Hospital, Oxford University Hospitals Birmingham NHS Foundation Trust	In follow-up	Phase I/II	2018	Mesenchymal stem/stromal cells	No	N/A	Allogeneic	Gastrointestinal (Liver)	Primary Sclerosing Cholangitis and Autoimmune Hepatitis
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 Healthcare NHS Trust University College London Hospitals NHS Foundation Trust University of Cambridge	In follow-up	Phase I	2012	Neural	No	N/A	Allogeneic	Neurological	Parkinson's Disease
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 General Infirmary	In follow-up	Phase II	2017	N/A	No	N/A	Autologous	Musculoskeletal	Bone Fracture
University of Oxford	The TWO study: Transplantation without Over-immunosuppression - A Phase IIb Trial of Regulatory T Cells in Renal Transplantation	EudraCT-2017-001421-41 ISRCTN11038572	Churchill Hospital, Oxford	Recruiting	Phase II	2018	T cells	No	N/A	Autologous	Inflammatory and immune system	Kidney Transplant Rejection

Cell and Gene Therapy Catapult ATMP Clinical Trials Database 2021

Sponsor	Title	Clinical database Numbers	United Kingdom Site(s)	Status	Phase	Year Started	Cell type	Gene modification / gene therapy	Type of Vector Used (if applicable)	Autologous/ Allogeneic	Disease Area	Indication
University of Oxford	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 EudraCT-2015-001383-18 ISRCTN15602229	Moorfields Eye Hospital NHS Foundation Trust London Oxford University Hospitals NHS Foundation Trust	Completed	Phase II	2015	N/A	Yes, in vivo	AAV	N/A	Ophthalmology	Choroideremia
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 EudraCT-2019-003884-23	Unknown UK site(s)	Withdrawn	Phase III	2020	N/A	Yes, in vivo	AAV	N/A	Oncology (other)	Ovarian Cancer
Vertex Pharmaceuticals Inc	A Phase I/II/III 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-Thalassemia	NCT03655678 EudraCT-2017-003351-38	Imperial College Healthcare NHS Trust	In follow-up	Phase I/II/III	2018	CD34 and/or CD133 stem cells	Yes, ex vivo	Non-viral	Autologous	Haematological	Transfusion-Dependent β Thalassemia
Vertex Pharmaceuticals Inc	A Phase I/II/III 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 EudraCT-2018-001320-19	Imperial College Healthcare NHS Trust	In follow-up	Phase I/II/III	2018	CD34 and/or CD133 stem cells	Yes, ex vivo	Non-viral	Autologous	Haematological	Severe Sickle Cell Disease (SCD)
Vertex Pharmaceuticals Inc	A Long-term Follow-up Study of Subjects with beta-thalassemia or Sickle Cell Disease Treated with Autologous CRISPR-Cas9 Modified Hematopoietic Stem Cells (CTX001)	NCT04208529 EudraCT-2018-002935-88	Imperial College Healthcare NHS Trust	Recruiting	Phase II	2021	CD34 and/or CD133 stem cells	Yes, ex vivo	Non-viral	Autologous	Haematological	Various
Vivet Therapeutics SAS	A Phase I/II, Multicenter, Non-randomized, Open Label, Adaptive Design, 5-year Follow-up, Single Dose-escalation Study of VTX-801 in Adult Patients with Wilson's Disease	NCT04537377 EudraCT-2020-000963-22	Royal Surrey NHS Foundation Trust	Recruiting	Phase I/II	2021	N/A	Yes, in vivo	AAV	N/A	Metabolic (Other)	Wilson Disease