Cell and Gene Therapy Catapult ATMP Clinical Trials Database 2022

Sponsor	Title	Clinical Database Numbers	Trial Phase	Trial Status	Disease Area	Indication	Cell Type	Gene Modification / Gene Therapy	Type of Vector Used (if applicable)	Autologous/ Allogeneic	Year Trial Started	United Kingdom Site(s)
Abeona Therapeutics Inc	Phase I/II Gene Transfer Clinical Trial of rAAV9.CMV.hNAGLU for Mucopolysaccharidosis (MPS) IIIB	NCT03315182 EudraCT-2014-001411-39	Phase I/II	Terminated	Metabolic (Other)	Mucopolysaccharidosis III (MPS III) (Sanfilippo Syndrome)	N/A	Yes, *in vivo*	AAV	N/A	2017	Hammersmith Hospital, London Birmingham Heartlands Hospital, Birmingham
Achilles Therapeutics Plc	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	Phase I/II	Recruiting	Oncology (Other)	Metastatic or Recurrent Melanoma	T cells	No	N/A	Autologous	2019	The Christie NHS Foundation Trust, Manchester Freeman Hospital, Newcastle Upon Tyne Cambridge University Hospitals NHS Foundation Trust, Cambridge University College London Hospital, London Guys and St Thomas' NHS Foundation Trust, London The Royal Marsden NHS Foundation Trust, London University hospital Southampton NHS Foundation Trust, Southampton
Achilles Therapeutics Plc	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	Phase I/II	Recruiting	Oncology (Other)	Non-Small Cell Lung Cancer	T cells	No	N/A	Autologous	2021	Birmingham NHS Foundation Trust, Birmingham Cambridge University Hospitals NHS Foundation Trust, Cambridge The Christie NHS Foundation Trust, Manchester University College London Hospital, London University Hospital Southampton NHS Foundation Trust, Southampton Freeman Hospital, Newcastle The Leeds Teaching Hospitals NHS Trust, Leeds Guys and St Thomas' NHS Foundation Trust, London
Achilles Therapeutics Plc	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 EudraCT: 2020-003423-41	Phase II	In planning/ set-up	Oncology (Other)	Metastatic or Recurrent Melanoma Non-Small Cell Lung Cancer	T cells	No	N/A	Autologous	2021	University Hospitals Birmingham NHS Foundation Trust, Birmingham Cambridge University Hospitals NHS Foundation Trust, Cambridge The Leeds Teaching Hospitals NHS Trust, Leeds University College London Hospital (UCLH), London Guys and St Thomas' NHS Foundation Trust, London The Royal Marsden NHS Foundation Trust, London The Christie NHS Foundation Trust, Manchester Freeman Hospital, Newcastle Upon Tyne University Hospital Southampton NHS Foundation Trust, Southampton
Adaptimmune Therapeutics Plc	A Phase I/II Open Label Clinical Trial Evaluating the Safety and Anti-tumor Activity of Autologous T Cells Expressing Enhanced TCRs Specific for Alpha Fetoprotein (AFPC332T) in HLA-A2 Positive Subjects with Advanced Hepatocellular Carcinoma (HCC)or Other AFP Expressing Tumor Types	NCT03132792	Phase I	In follow-up	Oncology (Other)	Hepatocellular Carcinoma Liver Cancer	T cells	Yes,	Lentivirus	Autologous	2017	Beatson West of Scotland Cancer Centre, Glasgow Guy's and St Thomas' NHS Foundation Trust, London UCLH Clinical Research Facility, london The Christie NHS Foundation Trust, Manchester
Adaptimmune Therapeutics Plc	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	Phase II	Recruiting	Oncology (Other)	Synovial Sarcoma, Myxoid/round cell liposarcoma	T cells	Yes, *ex vivo*	Lentivirus	Autologous	2020	University College London Hospital, London The Christie NHS Fouandation Trust, Manchester
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	Phase II	Recruiting	Oncology (Other)	Oesophageal Cancer	T cells	Yes, *ex vivo*	Lentivirus	Autologous	2021	The Christie NHS Foundation Trust, Withington, Manchester Beatson West of Scotland Cancer Centre, Glasgow University College Hospital, London Guy's Hospital-Guy's and St Thomas NHS Foundation Trust, London

Cell and Gene Therapy Catapult ATMP Clinical Trials Database 2022

Sponsor	Title	Clinical Database Numbers	Trial Phase	Trial Status	Disease Area	Indication	Cell Type	Gene Modification / Gene Therapy	Type of Vector Used (if applicable)	Autologous/ Allogeneic	Year Trial Started	United Kingdom Site(s)
AlloVir Inc	Phase 2/3, Multicenter, Randomized, Double-Blind, Placebo-Controlled Study to Assess the Safety and Efficacy of ALVR105 (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	EudraCT: 2021-005105-27	Phase II/III	In planning/ set-up	Infectious Disease	Treatment for 6 viral pathogens potentially transmitted through transplantation	T cells	No	N/A	Allogeneic	2022	Bristol Haematology and Oncology Centre, UHBW Bristol
AlloVir Inc	Phase II/III, Multicenter, Randomized, Double-Blind, Placebo-Controlled Study to Assess the Safety and Efficacy of ALVR105 (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	NCT05305040	Phase II/III	Recruiting	Infectious Disease	Treatment for 6 viral pathogens potentially transmitted through transplantation	T cells	No	N/A	Allogeneic	2022	Queen Elizabeth University Hospital, Glasgow Kings College Hospital, London Bristol Haematology and Oncology Centre, UHBW Bristol
AlloVir Inc	Phase III, Randomized, Double-Blind, Placebo-Controlled Trial, with Cross-Over, of Posoleucel (ALVR105) for the Treatment of Adenovirus Infection in Pediatric and Adult Participants Receiving Standard of Care Following Allogeneic Hematopoietic Cell Transplantation	NCT05179057 EudraCT-2021-003450-22	Phase III	In follow-up	Infectious Disease	Adenoviridae Infections	T cells	No	N/A	Allogeneic	2022	Great Ormond Street Hospital for Children, London Bristol Royal Hospital for Children, Bristol Royal Hospital for Children, Glasgow
AlloVir Inc	Global Registry for Long-term Follow-up of Patients Participating in Clinical Trials with Posoleucel (ALVR105)	NCT04693637 EudraCT-2022-000763-45 ISRCTN31439492	Phase IV	Recruiting	Infectious Disease	Treatment for 6 viral pathogens potentially transmitted through transplantation	T cells	No	N/A	Allogeneic	2022	Bristol Haematology and Oncology Centre, UHBW Bristol University College London Hospital, London Queen Elizabeth University Hospital, Glasgow Queen Elizabeth Hospital, Birmingham St. Mary's Hospital, London Royal Hospital for Sick Children, Glasgow Birmingham Childrens Hospital, Birmingham Kings College Hospital, London Bristol Royal Hospital for Children, Bristol Sheffield Children's NHS Foundation Trust, Sheffield Royal Manchester Childrens Hospital, Manchester Hammersmith Hospitals NHS Trust, London Great Ormond Street Hospital for Children, London Nottingham University Hospitals NHS Trust, Nottingham Royal Marsden Hospital, London
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	Phase III	Recruiting	Renal and Urogenital	Hemorrhagic Cystitis	T cells	No	N/A	Allogeneic	2022	Bristol Haematology and Oncology Centre, UHBW Bristol
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 EudraCT-2021-003450-22	Phase II	In follow-up	Infectious Disease	High-Risk Allogeneic Hematopoietic Cell Transplant patients	T cells	No	N/A	Allogeneic	2021	Unknown UK Site (s)
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	Phase IV	Recruiting	Oncology (Other)	Melanoma	N/A	Yes, *in vivo*	Oncolytic Virus	N/A	2017	Cheltenham General Hospital, Cheltenham Churchill Hospital, Oxford

Cell and Gene Therapy Catapult ATMP Clinical Trials Database 2022

Sponsor	Title	Clinical Database Numbers	Trial Phase	Trial Status	Disease Area	Indication	Cell Type	Gene Modification / Gene Therapy	Type of Vector Used (if applicable)	Autologous/ Allogeneic	Year Trial Started	United Kingdom Site(s)
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	Phase II	In follow-up	Oncology (Other)	Melanoma	N/A	Yes, *in vivo*	Oncolytic Virus	N/A	2020	Guys Hospital, London Royal Marsden Hospital, London
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	Phase II/III	In follow-up	Oncology (Other)	Malignant Pleural Mesothelioma Malignant Mesothelioma	Antigen present-ing cells	No	N/A	Autologous	2018	University of Leicester, Leicester
Astellas Gene Therapies	ASPIRO: A Phase I/II/III, 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	Phase I/II	In follow-up	Neuromuscular	X-Linked Myotubular Myopathy (XLMTM or MTM)	N/A	Yes, *in vivo*	AAV	N/A	2017	Great Ormond Street Hospital for Children, London
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	Phase I/II	In follow-up	Metabolic (Other)	Pompe Disease	N/A	Yes, *in vivo*	AAV	N/A	2020	The Newcastle upon Tyne Hospitals NHS Foundation Trust, Newcastle
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	Phase I/II	Recruiting	Ophthalmology	Age Related Macular Degeneration Dry (Atrophic) Macular Degeneration	Retinal	No	N/A	Allogeneic	2018	Undesclosed Site, London
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	Phase I	In follow-up	Oncology (Other)	Advanced Solid Tumors	N/A	Yes, *in vivo*	Oncolytic Virus	N/A	2019	Undisclosed Site, Leeds Undisclosed Site, London
Atamyo Therapeutics SAS	A Phase I/II Multicenter Study (2-Stages) to Evaluate the Safety and Efficacy of Intravenous GNT0006, Adeno-associated Viral Vector Carrying the FKRP Gene, in Patients with FKRP-related Limb-girdle Muscular Dystrophy (LGMDR9, Formerly LGMD2I)	NCT05224505 EudraCT-2021-004276-33	Phase I/II	Recruiting	Genetic Disorders	FKRP-related Limb-girdle Muscular Dystrophy (LGMDR9, Formerly LGMD2I)	N/A	Yes, *in vivo*	AAV	N/A	2022	Royal Victoria Infirmary, Newcastle Newcastle upon Tyne Hospitals NHS Foundation Trust, Newcastle

Cell and Gene Therapy Catapult ATMP Clinical Trials Database 2022

Sponsor	Title	Clinical Database Numbers	Trial Phase	Trial Status	Disease Area	Indication	Cell Type	Gene Modification / Gene Therapy	Type of Vector Used (if applicable)	Autologous/ Allogeneic	Year Trial Started	United Kingdom Site(s)
Atara Biotherapeutics Inc	An Open-label, Single-arm, Multicohort, Phase II Study to Assess the Efficacy and Safety of Tabelecleucel in Subjects with Epstein-barr Virus-associated Diseases	NCT04554914 EudraCT-2020-000177-25	Phase II	Recruiting	Infectious Disease	Epstein-Barr Virus-Associated Diseases	T cells	No	N/A	Allogeneic	2021	Great Ormond Street Hospital for Children NHS Trust, London University Hospital Birmingham NHS Foundation Trust, Birmingham
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	Phase III	In follow-up	Oncology (Other)	Epstein-Barr Virus-Associated Post-Transplant Lymphoproliferative Disease	T cells	No	N/A	Allogeneic	2020	Imperial College Healthcare NHS Trust, London Queen Elizabeth Hospital, Birmingham Kings College Hospital NHS Foundation Trust, London
Athersys Inc	MultiStem Administration for Stroke Treatment and Enhanced Recovery Study (MASTERS-2)	NCT03545607 EudraCT-2019-001680-69	Phase III	Recruiting	Cardiovascular	Acute Ischemic Stroke	Mesenchymal stem/stromal cells	No	N/A	Allogeneic	2021	Undisclosed UK sites
Autolus Therapeutics Plc	A Single Arm, Open Label, Multi-Center, 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	Phase I/II	Recruiting	Oncology (Haematology)	T-Cell Lymphomas Peripheral T-Cell Lymphomas (PTCL) Non-Hodgkin Lymphoma Angioimmunoblastic T-Cell Lymphoma (AITL)/ Immunoblastic Lymphadenopathy Anaplastic Large Cell Lymphoma (ALCL)	T cells	Yes, *ex vivo*	Retrovirus	Autologous	2018	The Christie NHS Foundation Trust, Manchester Freeman Hospital, Newcastle Manchester Royal Infirmary, Manchester University College London Hospital, London The Beatson West of Scotland Cancer Centre, Glasgow Newcastle Upon Tyne Hospital, Newcastle
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	Phase I/II	In follow-up	Oncology (Haematology)	B-Cell lymphoma	T cells	Yes, *ex vivo*	Retrovirus	Autologous	2017	Freeman Hospital, Newcastle upon Tyne The Christie NHS Foundation Trust, Manchester University College London Hospitals NHS Foundation Trust, London The Beatson West of Scotland Cancer Centre, Glasgow
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	Phase II	Recruiting	Oncology (Other)	Multiple Myeloma	T cells	Yes, *ex vivo*	Retrovirus	Autologous	2018	Great Ormond Street Hospital NHS Foundation Trust, London University College London Hospital, London Royal Manchester Children's Hospital, Manchester Freeman Hospital, Newcastle
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	Phase I/II	Recruiting	Oncology (Haematology)	B-Cell Acute Lymphocytic Leukemia	T cells	Yes, *ex vivo*	Lentivirus	Autologous	2020	Bristol Haematology and Oncology Centre, UHBW Bristol Queen Elizabeth Hospital NHS Foundation Trus University College London Hospitals NHS Foundation Trust, London King's College Hospital, London Manchester Royal Infirmary Hospital, Manchester Freeman Hospital, Newcastle upon Tyne Beatson West of Scotland Cancer Center, Glasgow

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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	Phase I/II	In follow-up	Haematological	Hemophilia A (Factor VIII Deficiency)	N/A	Yes, *in vivo*	AAV	N/A	2018	Manchester Royal Infirmary, Manchester
Belfast Health and Social Care Trust	Repair of Acute Respiratory Distress Syndrome by Stromal Cell Administration (REALIST): An Open Label Dose Escalation Phase I Trial Followed by a Randomized, Double-blind, Placebo-controlled Phase II Trial	NCT03042143 EudraCT-2017-000584-33	Phase I/II	Recruiting	Respiratory	Acute Respiratory Distress Syndrome (ARDS) due to COVID-19 or other causes of ARDS	Mesenchymal stem/stromal cells	No	N/A	Allogeneic	2019	Royal Hospitals, Belfast Health and Social Care Trust Guy's & St Thomas', Guy's and St Thomas' NHS Foundation Trust King's College Hospital, King's College Hospital NHS Foundation Trust Queen Elizabeth Hospital, University Hospital Birmingham NHS Foundation Trust Birmingham Heartlands, University Hospital Birmingham NHS Foundation Trust Edinburgh Royal Infirmary, Royal Infirmary of Edinburgh University College Hospital, University College London Hospital NHS Foundation Trust Manchester Royal Infirmary, Manchester University NHS Foundation Trust Sunderland Hospital, South Tyneside NHS Foundation Trust Liverpool Royal Infirmary, Liverpool University Hospitals NHS Foundation Trust Southampton Hospital, University Hospital Southampton NHS Foundation Trust Wythenshawe Hospital, Manchester University NHS Foundation Trust Aintree University Hospital, Liverpool University Hospitals NHS Foundation Trust
Bellicum Pharmaceuticals Inc	Phase II Extension Study of CaspaCIDe T Cells (BPX-501) From an HLA-partially Matched Family Donor after Negative Selection of TCR αβ+T Cells in Pediatric Patients Affected by Hematological Disorders	NCT02065869 EudraCT-2014-000584-41	Phase I/II	In follow-up	Oncology (Haema-tology)	Haematological Malignancies	T cells	Yes, *ex vivo*	Retrovirus	Allogeneic	2020	Great Ormond Street Hospital, London, Great North Children's Hospital, Newcastle Royal Free London NHS Foundation Trust, London Newcastle Upon Tyne Hospital, Newcastle
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	Phase I/II/III	Completed	Ophthalmology	Retinitis Pigmentosa (Retinitis)	N/A	Yes, *in vivo*	AAV	N/A	2017	Manchester Royal Eye Hospital, Manchester Oxford Eye Hospital, Oxford Undesclosed Site, Southampton,
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	Phase III	Recruiting	Ophthalmology	Choroideremia	N/A	Yes, *in vivo*	AAV	N/A	2018	Undisclosed Site, London Undisclosed Site, Manchester Undisclosed Site, Oxford Undisclosed Site, Southampton
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	Phase I/II	In follow-up	Haematological	Hemophilia A (Factor VIII Deficiency)	N/A	Yes, *in vivo*	AAV	N/A	2015	Hammersmith Hospital, London The Royal London Hospital, London Basingstoke and North Hampshire Hospital, Basingstoke Queen Elizabeth Hospital Birmingham, Birmingham St. Thomas' Hospital, London Royal Infirmary, Glasgow Addenbrooke's Hospital, Cambridge Bristol Haematology and Oncology Centre, UHBW Bristol University Hospital Southampton NHS Foundation Trust, Southampton
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	Phase III	Completed	Haematological	Haemophilia A	N/A	Yes, *in vivo*	AAV	N/A	2017	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, Southampton Royal Cornwall Hospital, Truro

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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	Phase I/II	Recruiting	Haematological	Hemophilia A (Factor VIII Deficiency)	N/A	Yes, *in vivo*	AAV	N/A	2018	Royal Free Hospital, London University Hospital Southampton NHS Foundation Trust, Southampton
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	GDC30021766	Phase I/II	In follow-up	Metabolic (Other)	Phenylketonuria (PKU)	N/A	Yes, *in vivo*	AAV	N/A	2020	University Hospital Birmingham NHS Foundation Trust, Birmingham
BioMarin Pharmaceutical Inc	A Phase I/II 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	Phase I/II	Recruiting	Haematological	Hemophilia A (Factor VIII Deficiency)	N/A	Yes, *in vivo*	AAV	N/A	2020	Royal Free Hospital, London Queen Elizabeth Hospital, Birmingham Guy's and St Thomas' NHS Foundation Trust, London
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	Phase III	Completed	Haematological	β-Thalassemia	CD34 and/or CD133 stem cells	Yes, *ex vivo*	Lentivirus	Autologous	2017	University College London Hospital, London
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	Phase III	Recruiting	Metabolic (Other)	Adrenoleukodystrophy	CD34 and/or CD133 stem cells	Yes, *ex vivo*	Lentivirus	Autologous	2015	Great Ormond Street Hospital for Children, London
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	Phase III	In follow-up	Haematological	β-Thalassemia	CD34 and/or CD133 stem cells	Yes, *ex vivo*	Lentivirus	Autologous	2017	University College London Hospital, London
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	Phase III	Recruiting	Haematological	Sickle Cell Disease	CD34 and/or CD133 stem cells	Yes, *ex vivo*	Lentivirus	Autologous	2022	Undisclosed UK sites

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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	Phase III	In follow-up	Metabolic (Other)	Adrenoleukodystrophy	CD34 and/or CD133 stem cells	Yes, *ex vivo*	Lentivirus	Autologous	2019	UCL Great Ormond Street Insitute of Child Health, London
bluebird bio Inc	Longterm Follow-up of Subjects With Transfusion-Dependent β-Thalassemia Treated With *Ex vivo* Gene Therapy Using Autologous Hematopoietic Stem Cells Transduced With a Lentiviral Vector	NCT02633943 EudraCT-2013-002245-11	Phase III	Recruiting	Haematological	Sickle Cell Disease β-Thalassemia	CD34 and/or CD133 stem cells	Yes, *ex vivo*	Lentivirus	Autologous	2013	University College London Hospital, London
Boston Children's Hospital	Multi-site Phase I/II Trial Evaluating the Treatment of SCID-X1 Patients With Retrovirus-mediated Gene Transfer	NCT01129544	Phase I/II	In follow-up	Inflammatory and immune system	X-Linked SCID Severe Combined Immune Deficiency (SCID)	CD34 and/or CD133 stem cells	Yes, *ex vivo*	Retrovirus	Autologous	2010	Great Ormond Street Hospital for Children, London
Boston Children's Hospital	Phase I/II Trial of Lentiviral Gene Transfer for SCID-X1 with Low Dose Targeted Busulfan Conditioning	NCT03311503	Phase I/II	Recruiting	Inflammatory and immune system	X-Linked SCID	CD34 and/or CD133 stem cells	Yes, *ex vivo*	Lentivirus	Autologous	2018	Great Ormond Street Hospital for Children, London
Bristol-Myers Squibb	A Study to Evaluate the Safety and Tolerability of BMS-986403 in Participants With Relapsed and/or Refractory Chronic Lymphocytic Leukemia or Small Lymphocytic Lymphoma	NCT05244070	Phase I	In planning/ set-up	Oncology (Haematology)	Relapsed and/or Refractory Chronic Lymphocytic Leukemia or Small Lymphocytic Lymphoma	T cells	Yes, *ex vivo*	Lentivirus	Autologous	2022	Cambridge University Hospitals NHS Foundation Trust, Cambridge Undisclosed site, Leeds Undisclosed site, London Undisclosed site, Manchester
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 IRAS: 297753	Phase I/II	In planning/ set-up	Neurological	Parkinson's Disease	Other	No	N/A	Allogeneic	2021	Cambridge University Hospitals NHS Foundation Trust, Cambridge
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 Advanced Non-small Cell Lung Cancer (NSCLC)	NCT03371485 EudraCT-2016-002577-35	Phase I	In follow-up	Oncology (Other)	Non-Small Cell Lung Cancer	Antigen presenting cells	No	N/A	Allogeneic	2018	Birmingham University Hospital, Birmingham Southampton General Hospital, Southampton Queen Elizabeth Hospital, Birmingham

Cell and Gene Therapy Catapult ATMP Clinical Trials Database 2022

Sponsor	Title	Clinical Database Numbers	Trial Phase	Trial Status	Disease Area	Indication	Cell Type	Gene Modification / Gene Therapy	Type of Vector Used (if applicable)	Autologous/ Allogeneic	Year Trial Started	United Kingdom Site(s)
Cancer Research UK	A Cancer Research UK Phase I/IIa trial of Chimpanzee Adenovirus Oxford 1 (CHADOX1) and Modified Vaccinia Ankara (MVA) vaccines against MAGE-A3 and NY-ESO-1 with standard of care treatment (chemotherapy and an immune checkpoint inhibitor)	NCT04908111 EudraCT2019-003015-64	Phase I/II	Recruiting	Oncology (Other)	Non-Small Cell Lung Cancer and Gastro-Oesophageal Cancer		Yes, *in vivo*	Adenovirus	N/A	2021	The Christie NHS Foundation Trust, Manchester St James' University Hospital, Leeds University Hospital Southampton The Beatson West of Scotland Cancer Centre, Glasgow Velindre University NHS Trust, Cardiff Guy's and St Thomas' NHS Foundation Trust, London Queen Elizabeth Hospital Birmingham
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	Phase III	In follow-up	Oncology (Haematology)	B-cell Non-Hodgkin Lymphomas	T cells	Yes, *ex vivo*	Lentivirus	Autologous	2018	UCL Cancer Institute, London University Hospital Southampton NHS Foundation Trust, Southampton
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	Phase III	In follow-up	Oncology (Haematology)	Multiple Myeloma	T cells	Yes, *ex vivo*	Lentivirus	Autologous	2019	The Leeds Teaching Hospitals NHS Trust, Leeds
Celgene Corp	Long-Term Follow-up Protocol for Subjects Treated With Gene-Modified T Cells	NCT03435796	Phase II/III	Recruiting	Oncology (Other)	Neoplasms	T cells	Yes, *ex vivo*	Lentivirus	Autologous	2019	University College London Hospital, London Southampton General Hospital, southampton Leeds Teaching Hospital, Leeds Kings College London, London The Christie Hospital, Manchester
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	Phase II	In follow-up	Oncology (Haematology)	B-Cell Non-Hodgkin Lymphoma	T cells	Yes, *ex vivo*	Lentivirus	Autologous	2018	UCL Cancer Institute, London The Christie NHS Foundation Trust, Manchester
Celgene Corp	A Phase II, Multi-Cohort, 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	Phase II	In follow-up	Oncology (Haematology)	Multiple Myeloma	T cells	Yes, *ex vivo*	Lentivirus	Autologous	2019	King's College Hospital, London
Celgene Corp	A 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	Phase II	Recruiting	Oncology (Haematology)	Follicular Lymphoma B-Cell Non-Hodgkin Lymphoma Marginal Zone B-cell Lymphoma Nodal Marginal Zone B-Cell Lymphoma	T cells	Yes, *ex vivo*	Lentivirus	Autologous	2020	The Christie NHS Foundation Trust, Manchester University College London Hospital, London

Cell and Gene Therapy Catapult ATMP Clinical Trials Database 2022

Sponsor	Title	Clinical Database Numbers	Trial Phase	Trial Status	Disease Area	Indication	Cell Type	Gene Modification / Gene Therapy	Type of Vector Used (if applicable)	Autologous/ Allogeneic	Year Trial Started	United Kingdom Site(s)
CellProthera SAS	Expanded Cell Endocardiac Transplantation (EXCELLENT)	NCT02669810 EudraCT 2014-001476-63	Phase I/II	Recruiting	Cardiovascular	Myocardial Infarction Congestive Heart Failure (Heart Failure)	CD34 and/or CD133 stem cells	No	N/A	Autologous	2015	Leeds Teaching Hospitals NHS Trust, Leeds Newcastle Upon Tyne Hospital, Newcastle, St Bartholomews Hospital, London Ninewells Hospital and Medical School, Dundee University of Edinburgh, Edinburgh Queen Elizabeth Hospital NHS Foundation Trust, King's Lynn
Celyad Oncology SA	An Open-label, Phase I Study to Assess the Safety of Multiple Doses of Cyad-101, Administered after Standard Folfox or Folfiri Chemotherapy in Patients with Unresectable Metastatic Colorectal Cancer	NCT03692429	Phase I	Recruiting	Oncology (Other)	Metastatic Colorectal Cancer	T cells	Yes, *ex vivo*	Retrovirus	Allogeneic	2018	Guy's and St Thomas' NHS Foundation Trust, London The Christie NHS Foundation Trust, Manchester
Cook MyoSite Inc	CELLEBRATE: An Adaptive, Two-Stage, Double-Blind, Randomized, Controlled Trial Comparing the Safety and Efficacy of AMDC-USR With Placebo in Female Subjects With Persistent or Recurrent Stress Urinary Incontinence Following Surgical Treatment	NCT03104517 EudraCT-2017-000956-25	Phase III	Recruiting	Renal and Urogenital	Stress Urinary Incontinence	Skeletal Muscle	No	N/A	Autologous	2018	Hull Royal Infirmary, Hull St. James's University Hospital, Leeds Derriford Hospital, Plymouth
CSL Ltd	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	Phase III	In follow-up	Haematological	Haemophilia B	N/A	Yes, *in vivo*	AAV	N/A	2018	University Hospitals Bristol NHS Foundation Trust, Bristol Addenbrooke's Hospital, Cambridge, The Royal London Hospital, London University Hospital Southampton NHS Foundation Trust, Southampton
Freeline Therapeutics Holdings Plc	A Phase 1/2, Open-label, Safety, Tolerability, and Efficacy Study of FLT201 in Adult Patients with Gaucher Disease Type 1 (GALILEO-1)	NCT05324943	Phase I/II	Recruiting	Genetic Disorders	Gaucher Disease Type 1	N/A	Yes, *in vivo*	AAV	N/A	2022	Queen Elizabeth Hospital, Birmingham Royal Free Hospital, London Salford Royal Hospital, Salford
Freeline Therapeutics Holdings Plc	A Dose Confirmation Study of FLT180a (Adeno-associated Viral Vector Containing the Padua Variant of a Codon-optimized Human Factor IX Gene) in Adult Subjects with Hemophilia B	NCT05164471 EudraCT-2021-001079-18	Phase I/II	Completed	Genetic Disorders	Hemophilia B	N/A	Yes, *in vivo*	AAV	N/A	2021	Glasgow Royal Infirmary, Glasgow Guys Hospital, London Royal Free London NHS Foundation Tust, London, Royal Victoria Infirmary, Newcastle
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	Phase I/II	In follow-up	Haematological	Haemophilia B	N/A	Yes, *in vivo*	AAV	N/A	2018	Royal Free London NHS Foundation Trust, London Oxford University Hospitals NHS Foundation Trust, Oxford

Cell and Gene Therapy Catapult ATMP Clinical Trials Database 2022

Sponsor	Title	Clinical Database Numbers	Trial Phase	Trial Status	Disease Area	Indication	Cell Type	Gene Modification / Gene Therapy	Type of Vector Used (if applicable)	Autologous/ Allogeneic	Year Trial Started	United Kingdom Site(s)
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	Phase I/II	Recruiting	Metabolic (Other)	Fabry Disease - a Lysosomal Storage Disorder	N/A	Yes, *in vivo*	AAV	N/A	2019	Royal Free Hospital, London
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	Phase II	Recruiting	Metabolic (Other)	Fabry Disease Lysosomal Storage Disorders	N/A	Yes, *in vivo*	AAV	N/A	2020	Royal Free London, London
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	Phase I/II	In follow-up	Inflammatory and immune system	Chronic Granulomatous Disease	CD34 and/or CD133 stem cells	Yes, *ex vivo*	Lentivirus	Autologous	2013	Great Ormond Street Hospital for Children NHS Trust, London Royal Free London NHS Foundation Trust, London University College London Hospital, London
Genethon SA	Long Term Safety Follow up of Patients Enrolled in the Phase I/II Clinical Trial of Haematopoietic Stem Cell Gene Therapy for the Wiskott-aldrich Syndrome (GTG 002-07 and GTG 003-08)	NCT02333760 EudraCT-2014-000274-20	Phase I/II	In follow-up	Inflammatory and immune system	Wiskott-Aldrich Syndrome	CD34 and/or CD133 stem cells	Yes, *ex vivo*	Lentivirus	Autologous	2014	UCL Institute of Child Health, London
Genethon SA	Phase I Single-arm, Dose-escalation Study of Microdystrophin Gene Transfer in Subjects with Duchenne Muscular Dystrophy (DMD)	EudraCT-2020-002093-27	Phase I/II/III	In follow-up	Neuromuscular	Duchenne Muscular Dystrophy	N/A	Yes, *in vivo*	AAV	N/A	2021	Undisclosed UK sites
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	Phase I/II	Recruiting	Ophthalmology	Retinitis Pigmentosa (Retinitis)	N/A	Yes, *in vivo*	AAV	N/A	2017	Moorfields Eye Hospital NHS Foundation Trust, London
GenSight Biologics SA	Efficacy 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	Phase III	In follow-up	Ophthalmology	Leber Hereditary Optic Neuropathy	N/A	Yes, *in vivo*	AAV	N/A	2018	Moorfields Eye Hospital NHS Foundation Trust, London

Cell and Gene Therapy Catapult ATMP Clinical Trials Database 2022

Sponsor	Title	Clinical Database Numbers	Trial Phase	Trial Status	Disease Area	Indication	Cell Type	Gene Modification / Gene Therapy	Type of Vector Used (if applicable)	Autologous/ Allogeneic	Year Trial Started	United Kingdom Site(s)
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 (RESTORE)	EudraCT-2017-002153-11 NCT03406104	Phase III	Completed	Ophthalmology	Leber Hereditary Optic Neuropathy	N/A	Yes, *in vivo*	AAV	N/A	2018	Moorfields Eye Hospital NHS Foundation Trust London
Gilead Sciences Inc	An Adaptive Phase III, Randomized, Open-Label, Multicenter Study to Compare the Efficacy and Safety of Axicabtagene Ciloleucel Versus Standard of Care Therapy as First-Line Therapy in Subjects With High-Risk Large B-Cell Lymphoma (ZUMA-23)	NCT05605899 EudraCT: 2022-501489-24-00	Phase III	In planning/ set-up	Oncology (Haematology)	Follicular Lymphoma, Diffuse Large B-Cell Lymphoma, Marginal Zone B-cell Lymphoma	T cells	Yes, *ex vivo*	Retrovirus	Autologous	2022	Cambridge University Hospitals NHS Foundation Trust, Cambridge
Gilead Sciences Inc	Long-term Follow-up Study for Participants of Kite-sponsored Interventional Studies Treated with Gene-modified Cells	NCT05041309 EudraCT-2020-005843-21	Phase II	Recruiting	Oncology (Haematology)	Refractory Large B-Cell Lymphoma	T cells	Yes, *ex vivo*	Retrovirus	Autologous	2021	Undisclosed UK site(s)
Gilead Sciences Inc	Long-term, Non-interventional Study of Recipients of Tecartus for Treatment of Adult Patients with Relapsed or Refractory Mantle Cell Lymphoma (MCL)	EUPAS45813	Phase IV	In planning/ set-up	Oncology (Haematology)	Mantle Cell Lymphoma	T cells	Yes, *ex vivo*	Retrovirus	Autologous	2022	Undisclosed UK site(s)
Great Ormond Street Hospital for Children NHS Trust	Phase I Study of Base Edited Car7 T Cells to Treat T Cell Malignancies (TvT CAR7)	NCT05397184 ISRCTN15323014 EudraCT: 2021-004312-25	Phase I	Recruiting	Oncology (Haematology)	T-cell leukaemia	T cells	No	N/A	Allogeneic	2022	Great Ormond Street Hospital for Children NHS Trust, London
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	Phase I/II	Recruiting	Inflammatory and immune system	X-Linked Severe Combined Immune Deficiency (SCID)	CD34 and/or CD133 stem cells	Yes, *ex vivo*	Lentivirus	Autologous	2018	Great Ormond Street Hospital NHS Trust London, London
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	Phase I/II	In follow-up	Inflammatory and immune system	Severe Combined Immunodeficiency Due to ADA Deficiency	CD34 and/or CD133 stem cells	Yes, *ex vivo*	Lentivirus	Autologous	2017	Great Ormond Street Hospital NHS Trust, London

Cell and Gene Therapy Catapult ATMP Clinical Trials Database 2022

Sponsor	Title	Clinical Database Numbers	Trial Phase	Trial Status	Disease Area	Indication	Cell Type	Gene Modification / Gene Therapy	Type of Vector Used (if applicable)	Autologous/ Allogeneic	Year Trial Started	United Kingdom Site(s)
Great Ormond Street Hospital for Children NHS Trust	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	Phase I	Recruiting	Oncology (Haema-tology)	B-Cell Acute Lymphocytic Leukemia (B-Cell Acute Lymphoblastic Leukaemia)	T cells	Yes, *ex vivo*	Lentivirus	Allogeneic	2020	Great Ormond Street Hospital for Children, London
GSK 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	Phase II	In follow-up	Oncology (Other)	Synovial Sarcoma Solid Tumor	T cells	Yes, *ex vivo*	Lentivirus	Autologous	2019	Undisclosed Site, London The Chrisite NHS Foundation Trust, Manchester
GSK plc	Long-Term Follow-Up (LTFU) of Participants Treated With GSK Adoptive Cell Therapies	NCT03391778 EudraCT-2018-004888-31	Phase I	Recruiting	Oncology (Other)	Various	T cells	Yes, *ex vivo*	Lentivirus	Autologous	2018	Undisclosed Site, London Undisclosed Site, Manchester Undisclosed Site, Sutton
GSK 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	Phase I/II	In follow-up	Oncology (Other)	Non-Small Cell Lung Cancer	T cells	Yes, *ex vivo*	Lentivirus	Autologous	2019	Undisclosed Site, London The Christie NHS Foundation Trust, Manchester
Gyroscope Therapeutics Holdings Ltd	Focus: An Open Label First in Human Phase I/II Multicentre Study to Evaluate the Safety, Dose Response and Efficacy ofGT005 Administered as a Single Subretinal Injection in Subjects with Macular Atrophy Due to AMD	NCT03846193 EudraCT-2017-003712-39	Phase I/II	In follow-up	Ophthalmology	Age-related Macular Degeneration (AMD)	N/A	Yes, *in vivo*	AAV	N/A	2018	Southampton General Hospital, Southampton, London Vision Clinic, London Moorfields Eye Hospital, London Sunderland Eye Infirmary, Sunderland Manchester Eye Hospital, Manchester Bristol Eye Hospital, UHBW Bristol Oxford University Hospital, Oxford
Gyroscope Therapeutics Holdings 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	Phase II	Recruiting	Ophthalmology	Age-related Macular Degeneration	N/A	Yes, *in vivo*	AAV	N/A	2020	Sunderland Eye Infirmary, Sunderland Bristol Eye Hospital, UHBW Bristol
Gyroscope Therapeutics Holdings 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	Phase II	Recruiting	Ophthalmology	Age Related Macular Degeneration	N/A	Yes, *in vivo*	AAV	N/A	2020	Sunderland Eye Infirmary, Sunderland Moorfileds Eye Hosiptial, London John Radcliff Hospital, Oxford London Vision Clinic, London

Cell and Gene Therapy Catapult ATMP Clinical Trials Database 2022

Sponsor	Title	Clinical Database Numbers	Trial Phase	Trial Status	Disease Area	Indication	Cell Type	Gene Modification / Gene Therapy	Type of Vector Used (if applicable)	Autologous/ Allogeneic	Year Trial Started	United Kingdom Site(s)
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	Phase IV	Completed	Ophthalmology	Burns Unspecified Ophthalmological Disorders	Corneal	No	N/A	Autologous	2018	Newcastle upon Tyne Hospitals NHS Foundation Trust, Newcastle Moorfields Eye Hospital, London Queen's Medical Centre, Nottingham
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	Phase IV	Recruiting	Ophthalmology	Burns Unspecified Ophthalmological Disorders	Corneal	No	N/A	Autologous	2016	Newcastle upon Tyne Hospitals NHS Foundation Trust, Newcastle Moorfields Eye Hospital, London
Hospital of the University of Munich	Treatment of Chemo-refractory Viral Infections after Allogeneic stem Cell Transplantation with Multispecific T Cells Against CMV, EBV and ADV: A Phase III, Prospective, Multicentre Clinical Trial	NCT04832607 EudraCT-2018-000853-29	Phase III	Recruiting	Infectious Disease	Cytomegalovirus (HHV-5) Infections Epstein–Barr Virus (HHV-4) Infections Adenoviridae Infections	T cells	No	N/A	Allogeneic	2019	Great North Children's Hospital, Newcastle upon Tyne
Imperial College London	Pancreatic Islet Transplantation to the Anterior Chamber of the Eye	NCT04198350	Phase II	Suspended	Metabolic (Diabetes)	Type 1 Diabetes (Juvenile Diabetes) Unspecified Ophthalmological Disorders	Pancreatic islets	No	N/A	Autologous	2022	Imperial College Healthcare NHS Trust, London
Innovacell Biotechnologie 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	Phase III	Recruiting	Gastrointestinal (Other)	Fecal Incontinence	Unknown	No	N/A	Autologous	2022	Undisclosed UK site(s)
Instil Bio Inc	A Phase II, Open-label, Multicenter Study Evaluating the Safety and Efficacy of Autologous Tumor-infiltrating Lymphocytes (TILs) in Subjects with Advanced Melanoma (DELTA-1)	NCT05050006 EudraCT-2020-003862-37	Phase II	Recruiting	Oncology (Other)	Advanced Melanoma	T cells	No	N/A	Autologous	2021	Cambridge University Hospitals NHS Foundation Trust, Cambridge
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	Phase I/II	Withdrawn	Oncology (Other)	Metastatic Ovarian Cancer	T cells	Yes, *ex vivo*	Unknown	Autologous	2020	Queens Elizabeth Hospital, Birmingham The Christies Hospital, Manchester

Cell and Gene Therapy Catapult ATMP Clinical Trials Database 2022

Sponsor	Title	Clinical Database Numbers	Trial Phase	Trial Status	Disease Area	Indication	Cell Type	Gene Modification / Gene Therapy	Type of Vector Used (if applicable)	Autologous/ Allogeneic	Year Trial Started	United Kingdom Site(s)
Intellia Therapeutics Inc	Phase I/II Study to Evaluate Safety, Tolerability, Pharmacokinetics, and Pharmacodynamics of NTLA-2002 in Adults with Hereditary Angioedema (HAE)	NCT05120830 EudraCT-2021-001693-33	Phase I/II	Recruiting	Genetic Disorders	Hereditary Angioedema (HAE) (C1 Esterase Inhibitor [C1-INH] Deficiency)	N/A	Yes, *in vivo*	Non-viral	N/A	2021	Cambridge University Hospitals NHS Foundation Trust, Cambridge
Intellia Therapeutics Inc		NCT04601051 EudraCT-2020-002034-32	Phase I	Recruiting	Metabolic (Other)	Hereditary Transthyretin Amyloidosis With Polyneuropathy (ATTRv-PN)	N/A	Yes, *in vivo*	Non-viral	N/A	2020	Undisclosed UK sites
Intellia Therapeutics Inc	Phase I/IIa, Single Dose Study Investigating NTLA-5001 in Subjects with Acute Myeloid Leukemia	NCT05066165	Phase I/II	Terminated	Oncology (Haema-tology)	AML	T cells	Yes, *ex vivo*	Unknown	Allogeneic	2021	Undisclosed site, Leeds Undisclosed site, London Undisclosed site, London Undisclosed site, Manchester
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	Phase II	Recruiting	Oncology (Other)	Cervical Carcinomas	T cells	No	N/A	Autologous	2017	Bristol Haematology and Oncology Centre, UHBW Bristol Sarah Cannon Research Institute, London University College London Hospitals NHS Foundation Trust, London NHS Greater Glasgow and Clyde, Glasgow
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	Phase II	Recruiting	Oncology (Other)	Metastatic Melanoma	T cells	No	N/A	Autologous	2019	Guy's Hospital, London Bristol Haematology and Oncology Centre, UHBW Bristol The Royal Marsden NHS Foundation Trust, London
Iovance Biotherapeutics Inc	A Phase II Multicenter Study of Autologous Tumor Infiltrating Lymphocytes (TIL or LN-145) in Patients With Metastatic Non-Small-Cell Lung Cancer	NCT04614103	Phase II	In planning/ set-up	Oncology (Other)	Non Small Cell Lung Cancer	T cells	No	N/A	Autologous	2022	Bristol Haematology and Oncology Centre, UHBW Bristol
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 EudraCT-2016-003980-21	Phase III	In follow-up	Cardiovascular	Critical Limb Ischemia in patients with Diabetes Mellitus	Bone marrow mononuclear cells	No	N/A	Autologous	2017	Addenbrooke's Hospital, Cambridge University Hospitals Birmingham NHS Foundation Trust,Birmingham University Hospital of Wales Cardiff, Freeman Hospital, Newcastle

Cell and Gene Therapy Catapult ATMP Clinical Trials Database 2022

Sponsor	Title	Clinical Database Numbers	Trial Phase	Trial Status	Disease Area	Indication	Cell Type	Gene Modification / Gene Therapy	Type of Vector Used (if applicable)	Autologous/ Allogeneic	Year Trial Started	United Kingdom Site(s)
Johnson & Johnson	A 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-2019-001413-16	Phase III	In follow-up	Oncology (Haematology)	Multiple Myeloma	T cells	Yes, *ex vivo*	Lentivirus	Autologous	2020	Queen Elizabeth Hospital, Birmingham Bristol Haematology and Oncology Centre, UHBW Bristol University College Hospital, London King's College Hospital, London Christie Hospital, Manchester Freeman Hospital, Newcastle Upon Tyne Cardiff and Vale University Local Health Board
Johnson & Johnson (Janssen)	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	NCT04923893 EudraCT-2021-001242-35	Phase III	Recruiting	Oncology (Haematology)	Multiple Myeloma	T cells	Yes, *ex vivo*	Lentivirus	Autologous	2022	University Hospitals Birmingham NHS Trust, Birmingham, Bristol Royal Infirmary, Bristol, Leeds Teaching Hospitals NHS Trust, Leeds, University College Hospital, London, King's College Hospital, London, Manchester Royal Infirmary, Manchester, The Royal Marsden NHS Trust Sutton, Surrey,
King's College London	Phase I Trial: T4 Immunotherapy of Head and Neck Cancer	NCT01818323 EudraCT-2012-001654-25 ISRCTN81726461	Phase I	Recruiting	Oncology (Other)	Head And Neck Cancer	T cells	Yes, *ex vivo*	Retrovirus	Autologous	2015	Guy's and St Thomas' NHS Foundation Trust, London
Kite Pharma Inc	A Phase II Multicenter Study Evaluating the Efficacy of KTE-X19 in Subjects With Relapsed/Refractory Mantle Cell Lymphoma	NCT04880434 EudraCT: 2015-005008-27	Phase II	Recruiting	Oncology (Haematology)	Mantle Cell Lymphoma	T cells	Yes, *ex vivo*	Retrovirus	Autologous	2021	Queen Elizabeth University Hospital, Glasgow Kings College Hospital, London Manchester Royal Infirmary, Manchester
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	Phase III	In follow-up	Oncology (Haematology)	B-Cell Lymphoma	T cells	Yes, *ex vivo*	Retrovirus	Autologous	2018	University College London Hospital, London The Christie NHS Foundation Trust, Manchester Barts Health NHS Trust, London University Hospitals Birmingham NHS Foundation Trust, Birmingham The Royal Marsden NHS Foundation Trust, Sutton
Kite Pharma Inc	A Phase I Multicenter Study Evaluating the Safety and Tolerability of KTE-X19 in Adult Subjects with Relapsed/Refractory Chronic Lymphocytic Leukemia and Small Lymphocytic Lymphoma	NCT03624036 EudraCT-2018-001923-38	Phase I	In follow-up	Oncology (Other)	Refractory Chronic Lymphocytic Leukemia (CLL)	T cells	Yes, *ex vivo*	Retrovirus	Autologous	2019	Cambridge University Hospitals NHS Foundation Trust, Cambridge
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	Phase II	Recruiting	Cardiovascular	Angina (Angina Pectoris) Coronary Artery Disease (CAD) (Ischemic Heart Disease) Refractory Angina	N/A	Yes, *in vivo*	Adenovirus	N/A	2019	Queen Mary University of London, London

Cell and Gene Therapy Catapult ATMP Clinical Trials Database 2022

Sponsor	Title	Clinical Database Numbers	Trial Phase	Trial Status	Disease Area	Indication	Cell Type	Gene Modification / Gene Therapy	Type of Vector Used (if applicable)	Autologous/ Allogeneic	Year Trial Started	United Kingdom Site(s)
Les Laboratoires Servier SAS	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	Phase I	In follow-up	Oncology (Haema-tology)	Advanced Lymphoid Malignancies	T cells	Yes, *ex vivo*	Lentivirus	Allogeneic	2016	The Christie NHS Foundation Trust, Manchester King's College Hospital NHS Foundation Trust, London Great Ormond Street Hospital for Children, London
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	Phase I/II	In follow-up	Metabolic (Other)	GM1 Gangliosidosis	N/A	Yes, *in vivo*	AAV	N/A	2021	Manchester University NHS Foundation Trust, Manchester
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	Phase II/III	In follow-up	Metabolic (Other)	Mucopolysaccharidosis Type IIIA	N/A	Yes, *in vivo*	AAV	N/A	2022	Great Ormond Street Hospital, London
Mario Negri Institute for Pharmacological Research	Novel Stromal Cell Therapy for Diabetic Kidney Disease (NEPHSTROM Study)	NCT02585622 EudraCT -2016-000661-23	Phase I/II	In follow-up	Renal and Urogenital	Type 2 Diabetes Diabetic Nephropathy	Mesenchymal stem/stromal cells	No	N/A	Allogeneic	2017	Belfast Health and Social Care Trust, Belfast University Hospital Birmingham NHS Foundation Trust, Birmingham
MeiraGTx	A Study of Adeno-associated Virus Vector Serotype 8 (AAV8)- hCARp. Human Cyclic Nucleotide-gated Channel Beta 3 (hCNGB3) Gene Therapy for the Treatment of Achromatopsia Associated with Variants in the CNGB3 Gene	CR109098	Phase II	Planned	Genetic Disorders	Achromatopsia	N/A	Yes, *in vivo*	AAV	N/A	2022	Moorfields Eye Hospital Manchester Royal Eye Hospital
MeiraGTx Holdings Plc	Long-Term Follow-Up Study of Participants Following an Open Label, Multi-Centre, Phase I/II Dose Escalation Trial of an Adeno-Associated Virus Vector (AAV2/5-OPTIRPE65) for Gene Therapy of Adults and Children with Retinal Dystrophy Owing to Defects in RPE65 (LCA2)	NCT02946879 EudraCT-2016-000898-20	Phase I/II	In follow-up	Ophthalmology	Leber Congenital Amaurosis (LCA)	N/A	Yes, *in vivo*	AAV	N/A	2016	Moorfields Eye Hospital, London
MeiraGTx Holdings Plc	Long-term Follow-up Study of Participants Following an Open Label, Multi-centre, Phase I/II Dose Escalation Trial of a Recombinant Adeno-associated Virus Vector (AAV2/8-hCARp. hCNGB3 and AAV2/8-hG1.7p.coCNGA3) for Gene Therapy of Adults and Children with Achromatopsia Owing to Defects in CNGB3 or CNGA3	NCT03278873 EudraCT-2016-003856-59	Phase I/II	In follow-up	Ophthalmology	Achromatopsia	N/A	Yes, *in vivo*	AAV	N/A	2017	Moorfields Eye Hospital NHS Foundation Trust, London

Cell and Gene Therapy Catapult ATMP Clinical Trials Database 2022

Sponsor	Title	Clinical Database Numbers	Trial Phase	Trial Status	Disease Area	Indication	Cell Type	Gene Modification / Gene Therapy	Type of Vector Used (if applicable)	Autologous/ Allogeneic	Year Trial Started	United Kingdom Site(s)
MeiraGTx Holdings Plc	Long Term Follow-Up 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	Phase I/II	Recruiting	Ophthalmology	X-Linked Retinitis Pigmentosa	N/A	Yes, *in vivo*	AAV	N/A	2018	Moorfields Eye Hospital NHS Foundation Trust London Leeds Teaching Hospitals NHS Trust, Leeds
MeiraGTx Holdings Plc	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	Phase III	Recruiting	Ophthalmology		X-Linked Retinitis Pigmentosa	N/A	Yes, *in vivo*	AAV	N/A	2021
MeiraGTx Holdings Plc	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	Phase III	Recruiting	Ophthalmology		X-Linked Retinitis Pigmentosa	N/A	Yes, *in vivo*	AAV	N/A	2022
Mina Therapeutics Ltd	A First-in-human, Multi-center, Open-label, Phase Ib/IIa Clinical Study with RNA Oligonucleotide Drug MTL-CEBPA to Investigate Its Safety and Tolerability in Patients with Advanced Liver Cancer	NCT02716012	Phase I	In follow-up	Oncology (Other)	Hepatocellular Carcinoma	N/A	Yes, *in vivo*	Non-viral	N/A	2016	University Hospitals Birmingham NHS Foundation Trust, Birmingham Cambridge University Hospitals NHS Trust, Cambridge Beatson West of Scotland Cancer Centre, Glasgow Clatterbridge Cancer Centre NHS Foundation Trust, Liverpool Guy's and St. Thomas' NHS Foundation Trust, London Imperial College Healthcare NHS Trust, London University College London Hospital, London Newcastle upon Tyne Hospitals NHS Foundation Trust, Newcastle
Moderna Therapeutics	A Study to Assess Safety, Pharmacokinetics, and Pharmacodynamics of mRNA-3705 in Participants With Isolated Methylmalonic Acidemia	NCT04899310	Phase I/II	Recruiting	Metabolic disease	Isolated Methylmalonic Acidemia Due to Methylmalonyl-CoA Mutase Deficiency	N/A	Yes, *in vivo*	Non-viral	N/A	2021	Royal Manchester Children's Hospital, Manchester Birmingham Childrens Hospital NHS foundation Trust, Birmingham
Montpellier University Hospital	A 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	Phase II	In follow-up	Musculoskeletal	Osteoarthritis (knee)	Mesenchymal stem/stromal cells	No	N/A	Autologous	2016	Cambridge University Hospitals NHS Foundation Trust, Cambridge
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	Phase I	Recruiting	Ophthalmology	Acute Wet Age Related Macular Degeneration	Retinal	No	N/A	Allogeneic	2016	Moorfields Eye Hospital, London

Cell and Gene Therapy Catapult ATMP Clinical Trials Database 2022

Sponsor	Title	Clinical Database Numbers	Trial Phase	Trial Status	Disease Area	Indication	Cell Type	Gene Modification / Gene Therapy	Type of Vector Used (if applicable)	Autologous/ Allogeneic	Year Trial Started	United Kingdom Site(s)
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	Phase III	In follow-up	Ophthalmology	Macular Telangectasia	Retinal	Yes, *ex vivo*	Non-viral	Allogeneic	2018	Oxford Eye Hospital, Oxford Moorfields Eye Hospital, London
Novartis AG	Long-term Follow-up of Patients With Spinal Muscular Atrophy Treated With OAV101 in Clinical Trials	NCT05335876	Phase III	In planning/ set-up	Neuromuscular	Spinal Muscular Atrophy	N/A	Yes, *in vivo*	AAV	N/A	2022	Newcastle upon Tyne Hospitals NHS FT, Newcastle
Novartis AG	Long Term Follow-Up of Patients Exposed to Lentiviral-Based CAR T-Cell Therapy	NCT02445222 EudraCT-2014-001673-14	N/A	Recruiting	Oncology (Haematology)	Unspecified B-Cell Lymphomas B-Cell Leukemia	T cells	Yes, *ex vivo*	Lentivirus	Autologous	2020	Undisclosed UK site(s)
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	Phase II	In follow-up	Oncology (Haematology)	Follicular Lymphoma	T cells	Yes, *ex vivo*	Lentivirus	Autologous	2018	Queen Elizabeth Hospital NHS Foundation Trust , Birmingham
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	Phase III	In follow-up	Oncology (Haematology)	Non-Hodgkin Lymphoma	T cells	Yes, *ex vivo*	Lentivirus	Autologous	2019	Queen Elizabeth Hospital, Birmingham University College Hospital, London
Novartis AG	A Phase II, Single Arm, Multicenter Open Label Trial to Determine the Safety and Efficacy of Tisagenlecleucel in Pediatric Subjects With Relapsed or Refractory Mature B-cell Non-Hodgkin Lymphoma (NHL) (BIANCA)#s3gt_translate_toolt-ip_mini { display: none !important; }	NCT03610724 EudraCT-2017-005019-15	Phase II	In follow-up	Oncology (Haematology)	B-Cell Non-Hodgkin Lymphoma	T cells	Yes, *ex vivo*	Lentivirus	Autologous	2019	Undisclosed sites Royal Manchester Children's Hospital, Manchester
Novartis AG	A Phase II Trial of Tisagenlecleucel in First-line High-risk (HR) Pediatric and Young Adult Patients with B-cell Acute Lymph-oblastic Leukemia (B-ALL) Who Are Minimal Residual Disease (MRD) Positive at the End of Consolidation (EOC) Therapy	NCT03876769 EudraCT-2017-002116-14	Phase II	Recruiting	Oncology (Haematology)	ALL, B-cell Leukaemia	T cells	Yes, *ex vivo*	Lentivirus	Autologous	2019	Undisclosed Site, London Undisclosed Site, London

Cell and Gene Therapy Catapult ATMP Clinical Trials Database 2022

Sponsor	Title	Clinical Database Numbers	Trial Phase	Trial Status	Disease Area	Indication	Cell Type	Gene Modification / Gene Therapy	Type of Vector Used (if applicable)	Autologous/ Allogeneic	Year Trial Started	United Kingdom Site(s)
Novartis AG	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	Phase III	In follow-up	Neuromuscular	Spinal Muscular Atrophy	N/A	Yes, *in vivo*	AAV	N/A	2021	Great North Children's Hospital, Newcastle
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	Phase IV	Recruiting	Neuromuscular	Spinal Muscular Atrophy (SMA)	N/A	Yes, *in vivo*	AAV	N/A	2020	Great Ormond Street Hospital for Children NHS Foundation Trust, London Newcastle Upon Tyne Hospitals NHS Foundation Trust, Newcastle
Ormond Medical Arts Pharmaceutical Research Center	Phase I/II, Non-randomised, Single-centre, Open-label Study of pCHIM-p47 (Lentiviral Vector Transduced CD34+ Cells) in Patients With p47 Autosomal Recessive Chronic Granulomatous Disease	NCT05207657	Phase I/II	In planning/ set-up	Haematological	Chronic Granulomatous Disease	CD34 and/or CD133 stem cells	Yes, *ex vivo*	Lentivirus	Autologous	2022	Great Ormond Street Hospital for Children NHS Trust, London
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	Phase I/II	Recruiting	Neurological	Krabbe Disease	N/A	Yes, *in vivo*	AAV	N/A	2022	Manchester University, Manchester
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	Phase I/II	Recruiting	Metabolic (Other)	GM1 Gangliosidosis	N/A	Yes, *in vivo*	AAV	N/A	2021	Great Ormond Street Hospital for Children, London
Passage Bio Inc	A Phase Ib/II 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	Phase I/II	Recruiting	Neurological	Frontotemporal Dementia	N/A	Yes, *in vivo*	AAV	N/A	2021	University of Cambridge, Cambridge
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	Phase III	Recruiting	Haematological	Hemophilia B (Factor IX Deficiency)	N/A	Yes, *in vivo*	AAV	N/A	2019	Newcastle upon Tyne Hospitals NHS FT, Newcastle Freeman Hospital, Newcastle Glasgow Royal Infirmary, Glasgow

Cell and Gene Therapy Catapult ATMP Clinical Trials Database 2022

Sponsor	Title	Clinical Database Numbers	Trial Phase	Trial Status	Disease Area	Indication	Cell Type	Gene Modification / Gene Therapy	Type of Vector Used (if applicable)	Autologous/ Allogeneic	Year Trial Started	United Kingdom Site(s)
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	Phase III	Recruiting	Neuromuscular	Duchenne Muscular Dystrophy	N/A	Yes, *in vivo*	AAV	N/A	2020	Great Ormond Street Hospital, London Royal Liverpool and Broadgreen University Hospitals NHS Trust, Liverpool Royal Victoria Infirmary, Newcastle Alder Hey Childrens NHS Foundation Trust, Liverpool
Pfizer Inc	Study to Evaluate the Efficacy and Safety of PF-07055480 / Giroctocogene Fitelparvovec Gene Therapy in Moderately Severe to Severe Hemophilia A Adults (AFFINE)	NCT04370054 EudraCT-2019-004451-37	Phase III	Recruiting	Haematological	Haemophilia A	N/A	Yes, *in vivo*	AAV	N/A	2020	Guy's and St. Thomas' NHS Foundation Trust London
Prevail Therapeutics Inc	An Open-label, Phase I/II Study to Evaluate the Safety and Efficacy of Single-dose LY3884961 in Infants With Type 2 Gaucher Disease	NCT04411654	Phase I/II	Recruiting	Metabolic (Other)	Lysosomal Storage Disorder (Type 2 Gaucher disease)	N/A	Yes, *in vivo*	AAV	N/A	2021	St Mary's Hospital, Manchester
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	Phase I/II	Recruiting	Neurological	Frontotemporal Dementia	N/A	Yes, *in vivo*	AAV	N/A	2020	University College London Hospital, London
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	Phase I	Recruiting	Oncology (Other)	Squamous Cell Carcinoma of the Head and Neck	N/A	Yes, *in vivo*	Oncolytic Virus	N/A	2021	Cardiff & Vale University LHB, Cardiff The Clatterbridge Cancer Centre, Liverpool The Royal Marsden Hospital, London University Hospital Southampton NHS Foundation Trust
Quell Therapeutics Ltd	A Single-arm, Open-label, Multi-center, Phase I/II Study Evaluating the Safety and Clinical Activity of QEL-001, an Autologous CAR T Regulatory Cell Treatment Targeting HLA-A2, in HLA-A2/ A28neg Patients That Have Received an HLA-A2pos Liver Transplant.	NCT05234190 EudraCT: 2021-001379-18	Phase I/II	Recruiting	Gastrointestinal (Liver)	Liver transplant rejection	T cells	Yes, *ex vivo*	Lentivirus	Autologous	2022	Cambridge University Hospitals NHS Foundation Trust, Cambridge Royal Free London NHS Foundation Trust, London King's College Hospital NHS Foundation Trust , London
ReNeuron Group Ltd.	A Phase I Safety Trial of CTX0E03 Drug Product Delivered Intracranially in the Treatment of Patients with Stable Ischemic Stroke	NCT01151124	Phase I	Completed	Cardiovascular	Stable Ischemic Stroke	Neural	Yes, *ex vivo*	Retrovirus	Allogeneic	2010	Southern General Hospital, Glasgow

Cell and Gene Therapy Catapult ATMP Clinical Trials Database 2022

Sponsor	Title	Clinical Database Numbers	Trial Phase	Trial Status	Disease Area	Indication	Cell Type	Gene Modification / Gene Therapy	Type of Vector Used (if applicable)	Autologous/ Allogeneic	Year Trial Started	United Kingdom Site(s)
Replimune Ltd	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	Phase I/II	Recruiting	Oncology (Other)	Melanoma	N/A	Yes, *in vivo*	Oncolytic Virus	N/A	2019	The Clatterbridge Cancer Centre NHS Foundation Trust, Bebington Oxford University Hospitals NHS Trust, Oxford University of Leeds- Teaching Hospital, Leeds Royal Marsden Hospital, London St James's Iniversity Hospital, Leeds Beatson West of Scotland Cancer Center, Glasgow
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	Phase II	Recruiting	Oncology (Other)	Cutaneous Squamous Cell Carcinoma	N/A	Yes, *in vivo*	Oncolytic Virus	N/A	2019	Undisclosed UK site(s)
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	Phase I	Recruiting	Oncology (Other)	Solid Tumor Triple-Negative Breast Cancer (TNBC)	N/A	Yes, *in vivo*	Oncolytic Virus	N/A	2019	The Clatterbridge Cancer Centre NHS Foundation Trust, Liverpool The Royal Marsden NHS Foundation Trust, London Churchill Hospital, Oxford, Royal Marsden Hospital, Sutton
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	Phase I	Recruiting	Oncology (Other)	Advanced Solid Tumor	N/A	Yes, *in vivo*	Oncolytic Virus	N/A	2020	The Clatterbridge Cancer Centre NHS Foundation Trust, Liverpool The Royal Marsden NHS Foundation Trust, London, Churchill Hospital, Oxford Royal Marsden Hospital, Sutton
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	Phase I/II	In follow-up	Dermatological	Epidermolysis Bullosa (EB)	Mesenchymal stem/stromal cells	No	N/A	Allogeneic	2018	Great Ormond Street Hospital for Children, London King's College London; St John's Institute of Dermatology, London
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-2018-002680-26	Phase I/II	In follow-up	Inflammatory and immune system	Leukocyte Adhesion Deficiency-I (LAD-I)	CD34 and/or CD133 stem cells	Yes, *ex vivo*	Lentivirus	Autologous	2020	Great Ormond Street Hospital for Children, London
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	Phase II	Recruiting	Haematological	Fanconi Anemia	CD34 and/or CD133 stem cells	Yes, *ex vivo*	Lentivirus	Autologous	2020	Great Ormond Street Hospital for Children, London

Cell and Gene Therapy Catapult ATMP Clinical Trials Database 2022

Sponsor	Title	Clinical Database Numbers	Trial Phase	Trial Status	Disease Area	Indication	Cell Type	Gene Modification / Gene Therapy	Type of Vector Used (if applicable)	Autologous/ Allogeneic	Year Trial Started	United Kingdom Site(s)
Sangamo Therapeutics Inc	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	Phase I/II	Recruiting	Metabolic (Other)	Fabry Disease	N/A	Yes, *in vivo*	AAV	N/A	2019	Queen Elizabeth NHS Foundation Trust, Kings Lynn Cambridge University Hospitals NHS Foundation Trust, Cambridge Royal Free Hospital, London
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	Phase I/II	Recruiting	Inflammatory and immune system	Kidney Transplant Rejection	T cells	Yes, *ex vivo*	Lentivirus	Autologous	2021	Oxford University Hospitals NHS Foundation Trust, Oxford
Sangamo Therapeutics Inc	Long-Term Follow-up of Fabry Disease Subjects Who Were Treated with ST-920, an AAV2/6 Human Alpha Galactosidase A Gene Therapy	NCT05039866	Phase I/II	Recruiting	Metabolic (Other)	Fabry Disease	N/A	Yes, *in vivo*	AAV	N/A	2021	Cambridge University Hospitals NHS Foundation Trust, Cambridge
Sarepta Therapeutics Inc	A Phase III Multinational, Randomized, Double-blind, Placebo-controlled Systemic Gene Delivery Study to Evaluate the Safety and Efficacy of SRP-9001 in Patients with Duchenne Muscular Dystrophy (Embark)	NCT05096221 EudraCT-2019-003374-91	Phase III	In follow-up	Genetic Disorders	Duchenne Muscular Dystrophy	N/A	Yes, *in vivo*	AAV	N/A	2021	Oxford University Hospitals NHS Foundation Trust, Oxford Great Ormond Street Hospital for Children NHS Foundation Trust, London The Newcastle Upon Tyne NHS Hospital Foundation Trust, Newcastle
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	Phase II	Recruiting	Oncology (Other)	Advanced unresectable melanoma	N/A	Yes, *in vivo*	Non-viral	N/A	2019	Velindre University NHS Trust, Cardiff East and North Hertfordshire NHS Trust, Northwood Nottingham University Hospitals NHS Trust, Nottingham Oxford University Hospital NHS Foundation Trust, Oxford
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	Phase I/II	Terminated	Haematological	Hemophilia A (Factor VIII Deficiency)	Retinal	Yes, *ex vivo*	Non-viral	Allogeneic	2020	Undisclosed Site, London Undisclosed Site, Southampton Manchester Royal Infirmary, Manchester
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	Phase I/II	Terminated	Neurological	Parkinson's Disease	N/A	Yes, *in vivo*	Lentivirus	N/A	2018	University of Cambridge, Centre for Brain Repair, Cambridge The National Hospital for Neurology and Neurosurgery, London University College London Hospital, London National Institute for Health Research, Southampton

Cell and Gene Therapy Catapult ATMP Clinical Trials Database 2022

Sponsor	Title	Clinical Database Numbers	Trial Phase	Trial Status	Disease Area	Indication	Cell Type	Gene Modification / Gene Therapy	Type of Vector Used (if applicable)	Autologous/ Allogeneic	Year Trial Started	United Kingdom Site(s)
Solid Biosciences Inc	A Randomized, Controlled, Open-label, Single-ascending Dose, Phase I/II Study to Investigate the Safety and Tolerability, and Efficacy of Intravenous SGT-001 in Male Adolescents and Children with Duchenne Muscular Dystrophy	NCT03368742 EudraCT-2017-002213-60	Phase I/II	In follow-up	Genetic Disorders	Duchenne Muscular Dystrophy	N/A	Yes, *in vivo*	AAV	N/A	2019	The Newcastle upon Tyne Hospitals NHS Foundation Trust, Newcastle
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	Phase I/II	In follow-up	Metabolic (Other)	Lysosomal Storage Disorder	N/A	Yes, *in vivo*	AAV	N/A	2020	Salford Royal MHS Foundation Trust, Salford The Royal Free London NHS Foundation Trust, london Queen Elizabeth Hospital, Birmingham
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	Phase I/II	In follow-up	Haematological	Hemophilia B (Factor IX Deficiency)	N/A	Yes, *in vivo*	AAV	N/A	2010	Royal Free Hospital, London
Takeda Pharmaceutical Co Ltd	An Observational Post-marketing Registry on the Effectiveness and Safety of Darvadstrocel in Patients with Crohn's Disease and Complex Perianal Fistulas (INSPIRE)	EUPAS24267	Phase IV	In follow-up	Gastrointestinal (Other)	Crohn's Disease and Complex Perianal Fistulas	Mesenchymal stem/stromal cells	No	N/A	Allogeneic	2018	St Mark's Hospital, London Northern General Hospital, Sheffield Sheffield Teaching Hospitals NHS Foundation Trust, Sheffield
TC BioPharm Ltd	ACHIEVE - An Adaptive Trial of the Efficacy and Effectiveness of Adoptive Cellular tHerapy with Ex-Vivo Expanded Allogeneic γδ T-lymphocytes (TCB-008) for Patients With Refractory or Relapsed Acute Myeloid Leukaemia (AML)	EudraCT-2021-000744-22	Phase II/III	In planning/ set-up	Oncology (Haema-tology)	Refactory/ Relapsed Acute Myeloid Leukaemia	T cells	No	N/A	Allogeneic	2022	Bristol Haematology and Oncology Centre, UHBW Bristol
TC BioPharm Ltd	A Phase II Safety and Tolerability, Inter-patient Pre-defined Dose Study of Ex-vivo Expanded Allogeneic gamma delta T-lymphocytes (TCB008) in Patients Diagnosed With COVID-19	NCT04834128	Phase II	In follow-up	Respiratory	Coronavirus Disease (COVID-19)	T cells	No	N/A	Allogeneic	2021	Newcastle upon Tyne Hospital NHS Foundation Trust, Newcastle
The Robert Jones & Agnes Hunt Orthopaedic Hospital NHS Foundation 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	Phase II	Recruiting	Musculoskeletal	Knee Osteoarthritis	Chondrocytes	No	N/A	Autologous	2013	The Robert Jones and Agnes Hunt Orthopaedic Hospital NHS Foundation Trust, Oswestry

Cell and Gene Therapy Catapult ATMP Clinical Trials Database 2022

Sponsor	Title	Clinical Database Numbers	Trial Phase	Trial Status	Disease Area	Indication	Cell Type	Gene Modification / Gene Therapy	Type of Vector Used (if applicable)	Autologous/ Allogeneic	Year Trial Started	United Kingdom Site(s)
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	Phase I/II	Recruiting	Neuromuscular	Duchenne Muscular Dystrophy	Other	Yes, *ex vivo*	Lentivirus	Autologous	2019	Royal Manchester Children's Hospital, Manchester
The University of Manchester Royal Manchester Children's Hospital	A Phase I/II, Study of Autologous CD34+ Haematopoietic Stem Cells Transduced *ex vivo* with CD11b Lentiviral Vector Encoding for Human SGSH in Patients With Mucopolysaccharidosis Type IIIA (MPS IIIa, Sanfilippo Syndrome Type A)	GDC40001081	Phase I/II	In follow-up	Metabolic (Other)	Mucopolysaccharidosis IIIA (MPS IIIA) (Sanfilip-po Syndrome) - Lysosomal storage disease	CD34 and/or CD133 stem cells	Yes, *ex vivo*	Lentivirus	Autologous	2020	Manchester University Hospital NHS Foundation Trust, Manchester
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	Phase III	In follow-up	Gastrointestinal (Other)	Crohn's Disease	Adipose-derived stem cells	No	N/A	Allogeneic	2019	Glasgow Royal Infirmary (GRI), Glasgow Addenbrooke's Hospital, Cambridge Royal Devon And Exeter Hospital, Exeter Wythenshawe Hospital, Manchester St. Mark's Hospital, London Guys & St Thomas, London
Transgene SA	A Dose-escalation and Phase I/IIa Study of TG6002 Plus Flucytosine in Patients with Unresectable Colorectal Cancer with Liver Metastases	NCT04194034 EudraCT-2018-004103-39	Phase I/II	Recruiting	Oncology (Other)	Colorectal Neoplasms	T cells	Yes, *in vivo*	Oncolytic Virus	N/A	2019	St James's University Hospital, Leeds
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	Phase I	Recruiting	Oncology (Other)	Squamous cell carcinoma of the head and neck	N/A	Yes, *in vivo*	MVA	N/A	2019	NHS Clatterbridge Cancer Center, Bebington Aintree University Hospital NHS Fondation Trust, Liverpool University Hospital NHS Fondation Trust, Southampton
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	Phase III	In follow-up	Oncology (Other)	Malignant Pleural Mesothelioma	N/A	Yes, *in vivo*	Adenovirus	N/A	2019	Beatson West of Scotland Cancer Centre, Glasgow Derriford Hospital, Plymouth Royal Marsden Foundation Trust, London Guy's and St. Thomas' NHS Trust, London Wythenshawe Hospital UHSM, Manchester Churchill Hospital, Oxford Queen Elizabeth Hospita,l Birmigham
Turnstone Biologics, Corp.	A Phase 1/2a, Multicenter, Open-label Trial of TBio-6517, an Oncolytic Vaccinia Virus, Administered by Intratumoral Injection, Alone and in Combination With Pembrolizumab, in Patients With Advanced Solid Tumors	NCT04301011 EudraCT-2021-001001-79	Phase I/II	Recruiting	Oncology (Other)	Solid Tumors	N/A	Yes, *in vivo*	Oncolytic Virus	N/A	2022	Unknown UK site(s)

Cell and Gene Therapy Catapult ATMP Clinical Trials Database 2022

Sponsor	Title	Clinical Database Numbers	Trial Phase	Trial Status	Disease Area	Indication	Cell Type	Gene Modification / Gene Therapy	Type of Vector Used (if applicable)	Autologous/ Allogeneic	Year Trial Started	United Kingdom Site(s)
Ultragenyx Pharmaceutical Inc	A Phase III, Randomized, Double-blind, Placebo-controlled Study of Adeno-associated Virus (AAV) Serotype 8 (AAV8)-Mediated Gene Transfer of Human Ornithine Transcarbamylase (OTC) in Patients With Late-onset OTC Deficiency	NCT05345171 EudraCT-2020-003384-25	Phase III	Recruiting	Genetic Disorders	Ornithine-Transcarbamylase Deficiency	N/A	Yes, *in vivo*	AAV	N/A	2022	University Hospitals Birmingham NHS Foundation Trust, Birmingham
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	Phase I/II	In follow-up	Metabolic (Other)	Ornithine-Transcarbamylase Deficiency	N/A	Yes, *in vivo*	AAV	N/A	2018	Queen Elizabeth Hospital, Birmingham
Unicyte AG	An Open-label, Non-controlled, Multi-center, Phase II Study to Assess the Efficacy and Safety of Allogeneic Human Liver Stem Cells (Hlscs) in the Treatment of Pediatric Patients With Early-onset Urea Cycle Disorder	EudraCT-2022-000933-18	Phase II	In planning/ set-up	Genetic Disorders	Urea cycle disordres	Other	No	N/A	Allogeneic	2022	Undisclosed UK site(s)
UniQure NV	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's Disease	EudraCT-2020-001461-36	Phase I/II	Recruiting	Neurological	Huntington Disease	N/A	Yes, *in vivo*	AAV	N/A	2021	University Hospital of Wales, Cardiff University College London Hospital, London
University College London	COBALT: Evaluation of CAR19 T-cells as an Optimal Bridge to Allogeneic Transplantation	NCT02431988 Eudra CT-2015-000348-40	Phase I	Completed	Oncology (Other)	Diffuse Large B-Cell Lymphoma	T cells	Yes, *ex vivo*	Lentivirus	Autologous	2016	University College London Hospital, London
University College London	CARPALL: Immunotherapy With CD19/22 CAR T-cells for CD19+ Haematological Malignancies	NCT02443831 EudraCT-2015-001144-10	Phase I	In follow-up	Oncology (Haematology)	ALL	T cells	Yes, *ex vivo*	Lentivirus	Autologous	2016	Royal Manchester Children's Hospital, Manchester Great Ormond Street Hospital for Children NHS Trust, London University College London Hospital, London Manchester Royal Infirmary, Manchester
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	Phase I	In follow-up	Oncology (Other)	CD19+ Malignancies	T cells	Yes, *ex vivo*	Unknown	Autologous	2017	University College London Hospital, London

Cell and Gene Therapy Catapult ATMP Clinical Trials Database 2022

Sponsor	Title	Clinical Database Numbers	Trial Phase	Trial Status	Disease Area	Indication	Cell Type	Gene Modification / Gene Therapy	Type of Vector Used (if applicable)	Autologous/ Allogeneic	Year Trial Started	United Kingdom Site(s)
University College London	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	Phase I/II	Recruiting	Oncology (Haema-tology)	Acute Lymphocytic Leukemia (ALL, Acute Lymphoblastic Leukemia) Lymphoma B-Cell Non-Hodgkin Lymphoma Chronic Lymphocytic Leukemia (CLL)	T cells	Yes, *ex vivo*	Lentivirus	Autologous	2017	University College London Hospital, London The Christie NHS Foundation Trust, Manchester The Beatson West of Scotland Cancer Centre, Glasgow
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	Phase I/II	Recruiting	Haematological	Hemophilia A (Factor VIII Deficiency)	N/A	Yes, *in vivo*	AAV	N/A	2017	Royal Free London NHS Foundation Trust, London
University College London	Immunotherapy with Tacrolimus Resistant EBV CTL for Lymphoproliferative Disease after Solid Organ Transplant	NCT03131934 EudraCT 2017-001020-22	Phase I	In follow-up	Oncology (Haema-tology)	Post-Transplant Lymphoproliferative Disorder	T cells	Yes, *ex vivo*	Retrovirus	Autologous	2019	Great Ormond Street Hospital for Children, London King's College Hospital, London
University College London	Targeted Stem Cells Expressing Trail as a Therapy for Lung Cancer	NCT03298763	Phase I/II	Recruiting	Oncology (Other)	Non-Small Cell Lung Cancer	Mesenchymal stem/stromal cells	Yes, *ex vivo*	Lentivirus	Allogeneic	2019	University College London Hospital, London
University College London	Phase I Study of Transfer of Effector Memory T Cells (Tem) Following Allogeneic Stem Cell Transplantation	NCT03836690	Phase I	Recruiting	Oncology (Haema-tology)	Lymphoma, Leukemia, Myeloma, Myelodys-plastic Syndromes, Severe Aplastic Anemia, Primary Immune Deficiency, Graft Vs Host Disease	T cells	No	N/A	Allogeneic	2019	University College London Hospital, London
University College London	Immunotherapy Using CAR T-cells to Target CD19 for Relapsed/Refractory CD19+ Primary Central Nervous System (CNS) Lymphoma	NCT04443829	Phase I	Recruiting	Oncology (Other)	Primary CNS Lymphoma	T cells	Yes, *ex vivo*	Unknown	Autologous	2021	University College London Hospital, London
University College London	An Open Label, Phase I Study Evaluating the Activity of Modular CAR T for Myeloma	NCT04795882	Phase I	Recruiting	Oncology (Haema-tology)	Multiple Myeloma	T cells	Yes, *ex vivo*	Lentivirus	Unknown	2022	University College London, London

Cell and Gene Therapy Catapult ATMP Clinical Trials Database 2022

Sponsor	Title	Clinical Database Numbers	Trial Phase	Trial Status	Disease Area	Indication	Cell Type	Gene Modification / Gene Therapy	Type of Vector Used (if applicable)	Autologous/ Allogeneic	Year Trial Started	United Kingdom Site(s)
University of Birmingham	A 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: 2019-003404-12 IRAS-272535	Phase II	In planning/ set-up	Gastrointestinal (Liver)	Lupus nephritis, PSC, Rheumatoid arthitis, crohns disease	Mesenchymal stem/stromal cells	No	N/A	Allogeneic	2020	University Hospitals Birmingham
University of California Los Angeles	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	Phase III	Recruiting	Inflammatory and immune system	Adenosine Deaminase (ADA) Deficiency Related SCID	CD34 and/or CD133 stem cells	Yes, *ex vivo*	Lentivirus	Autologous	2019	UCL Great Ormond Street Institute of Child Health, London
University of 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	Phase II	Completed	Musculoskeletal	Bone Fracture	N/A	No	N/A	Autologous	2016	Leeds General Infirmary, Leeds Hull Royal Infirmary, Hull
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	Phase II	Recruiting	Inflammatory and immune system	Kidney Transplant Rejection	T cells	No	N/A	Autologous	2018	University of Oxford, Oxford
Vertex Pharmaceuticals Inc	A Phase III Study to Evaluate the Safety and Efficacy of a Single Dose of CTX001 in Pediatric Subjects With Severe Sickle Cell Disease	NCT05329649 EudraCT-2021-002173-26	Phase III	Recruiting	Haematological	Severe Sickle Cell Disease (SCD)	CD34 and/or CD133 stem cells	Yes, *ex vivo*	N/A	Autologous	2022	Undisclosed UK site(s)
Vertex Pharmaceuticals Inc	A Phase III Study to Evaluate the Safety and Efficacy of a Single Dose of CTX001 in Pediatric Subjects With Transfusion-Dependent β-Thalassemia	NCT05356195 EudraCT-2021-002172-39	Phase III	Recruiting	Genetic Disorders	Transfusion-Dependent β-Thalassemia	CD34 and/or CD133 stem cells	Yes, *ex vivo*	N/A	Autologous	2022	Undisclosed UK site(s)
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	Phase I/II	In follow-up	Haematological	Transfusion-Dependent β Thalassemia	CD34 and/or CD133 stem cells	Yes, *ex vivo*	Non-viral	Autologous	2018	Imperial College Healthcare, London

Cell and Gene Therapy Catapult ATMP Clinical Trials Database 2022

Sponsor	Title	Clinical Database Numbers	Trial Phase	Trial Status	Disease Area	Indication	Cell Type	Gene Modification / Gene Therapy	Type of Vector Used (if applicable)	Autologous/ Allogeneic	Year Trial Started	United Kingdom Site(s)
Vertex Pharmaceuticals Inc	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	Phase II/III	In follow-up	Haematological	Severe Sickle Cell Disease (SCD)	CD34 and/or CD133 stem cells	Yes, *ex vivo*	Non-viral	Autologous	2018	Imperial College Healthcare NHS Trust, London Hammersmith Hospital, London
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	Phase II	Recruiting	Haematological	Multiple	CD34 and/or CD133 stem cells	Yes, *ex vivo*	Non-viral	Autologous	2021	Imperial College Healthcare NHS Trust, London Hammersmith Hospital, London
Videregen Ltd	Phase I/II, Open Label Study to Assess the Safety and Efficacy of a novel tissue engineered airway product, consisting of expanded Autologous Bone Marrow-derived Mesenchymal Stromal Cells Seeded on to a Decellularised allogeneic patch of an Airway Scaffold in Subjects with Clinically Significant Bronchopleural Fistula	NCT04435249 EudraCT-2019-004939-24	Phase I/II	In planning/ set-up	Respiratory	Bronchopleural Fistula	Mesenchymal stem/stromal cells	No	N/A	Autologous	2022	Royal Papworth Hospital Royal Free Hospital
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	Phase I/II	Recruiting	Metabolic (Other)	Wilson Disease	N/A	Yes, *in vivo*	AAV	N/A	2021	Royal Surrey County Hospital, Guilford