CATAPULT
Cell and Gene Therapy

Cell and Gene Therapy Catapult

UK 2024 ATMP Clinical Trials Database

This clinical trial database covers all ATMP trials taking place in the UK in 2024.

Ongoing trial numbers in the UK have increased (187 in 2024 vs 175 in 2023).

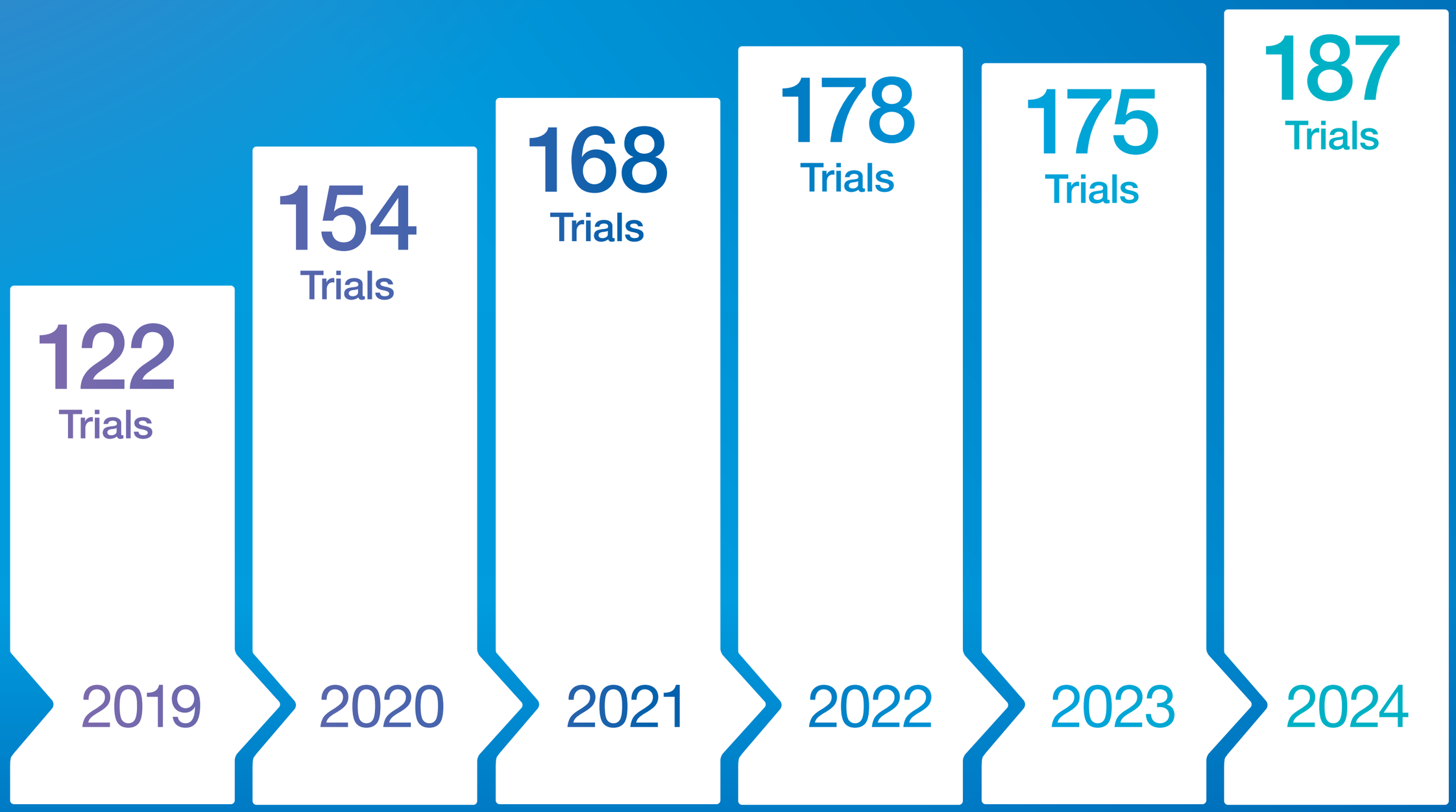
Here, we highlight key data from the database ▶

UK clinical trial capability remains strong

Ongoing clinical trials in the UK have increased by 7% compared to 2023.

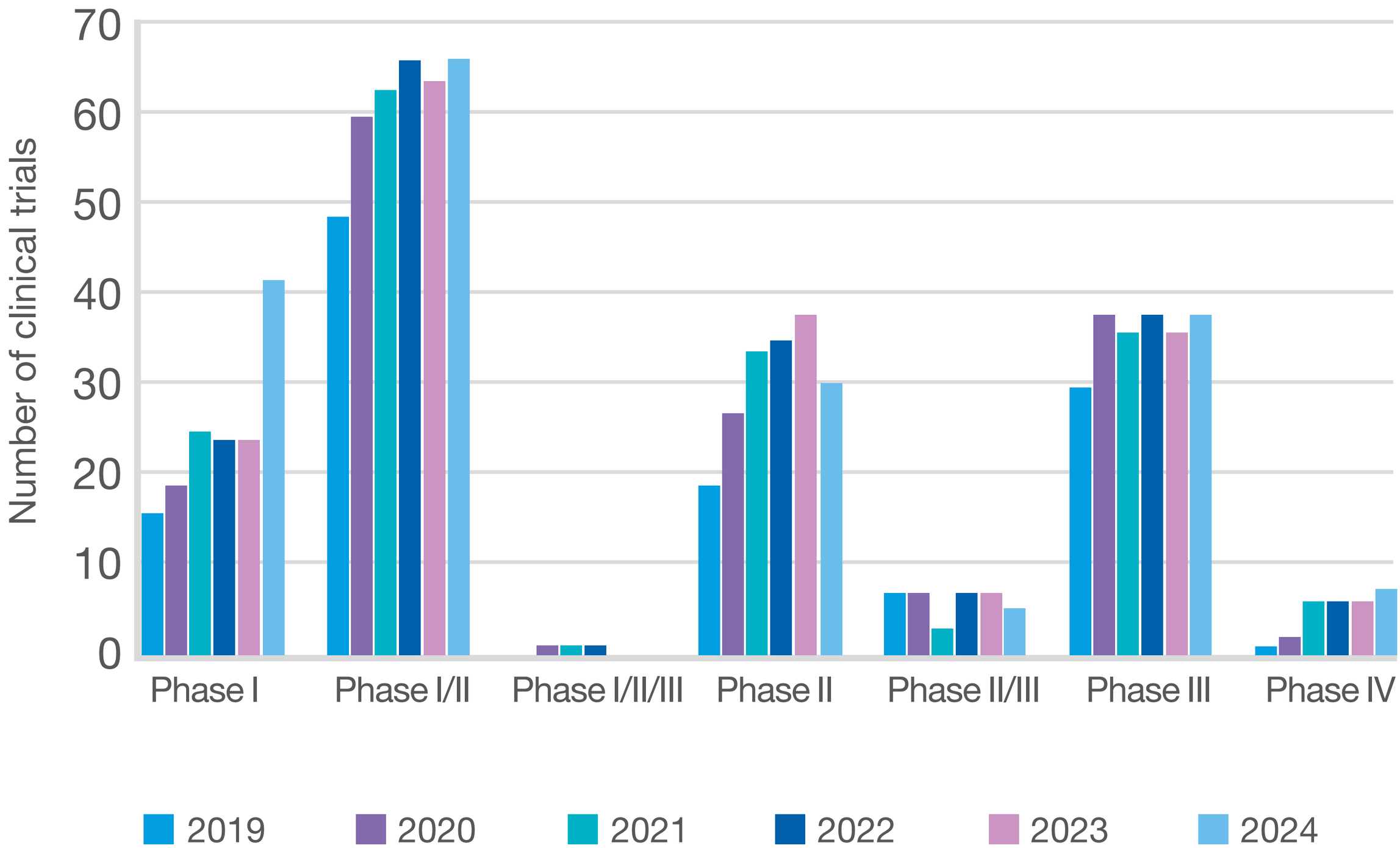
9.5%

of global trials have representation in the UK



70% growth in early-stage clinical trials

The number of Phase I trials have almost doubled, going from 24 to 41.

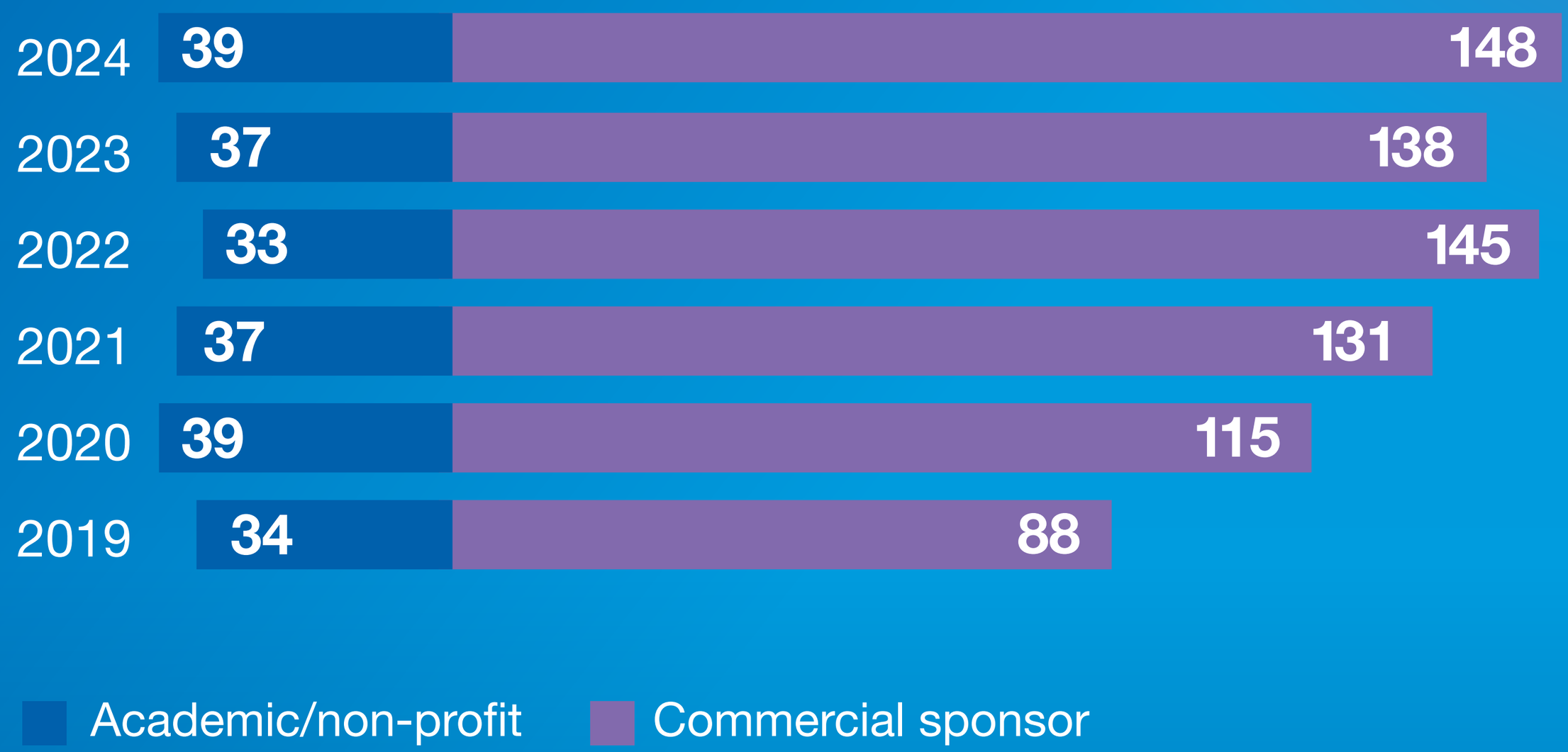


UK continues to be an attractive location for commercially sponsored ATMP trials

~80%

of trials are conducted by commercial sponsors

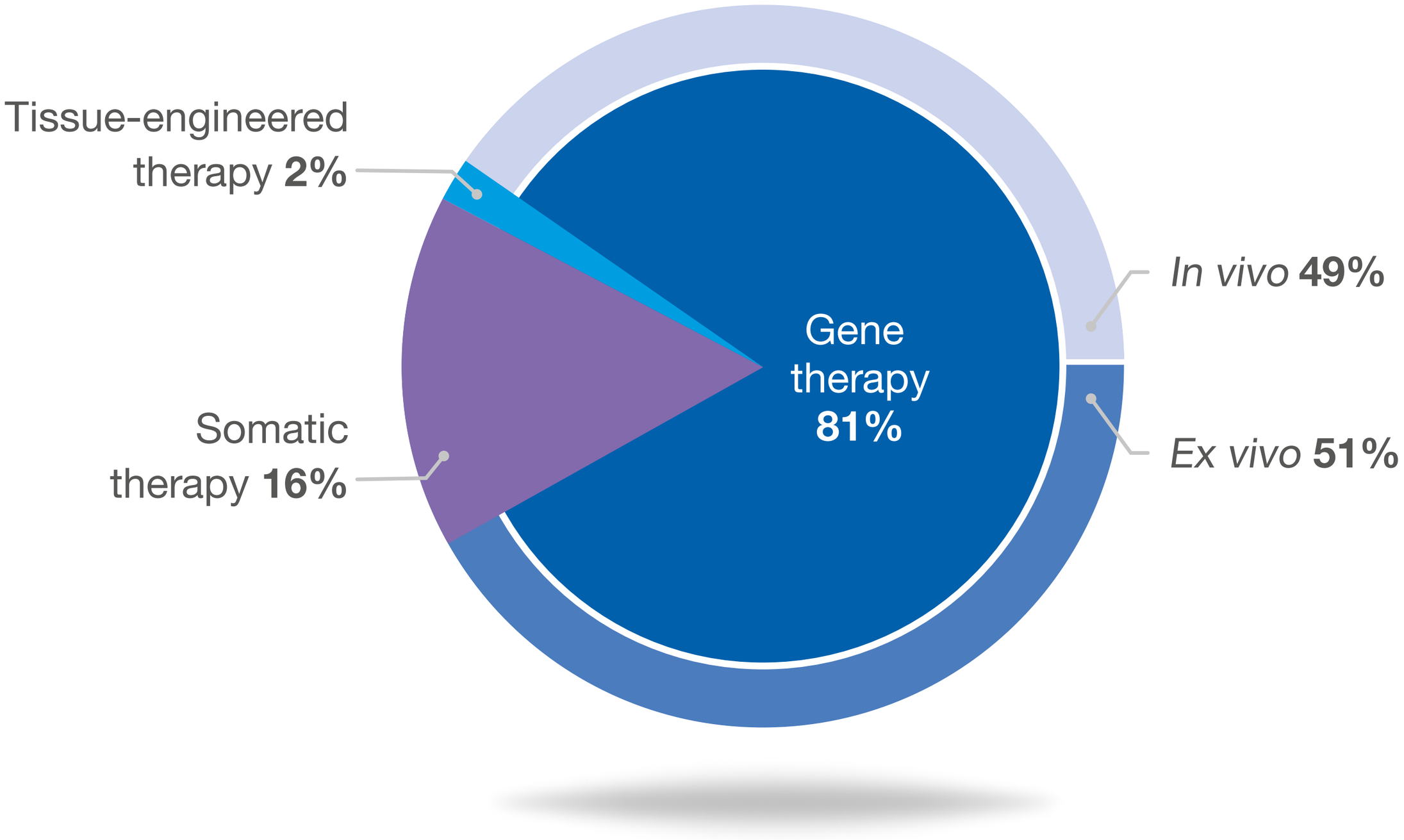
The proportion of clinical trials conducted by commercial sponsors is consistent with last year.



Data from ARM 2024 CT report and CGT Catapult 2023 ATMP Clinical Trials Database

In 2024, the majority of trials involve genetically modified therapies

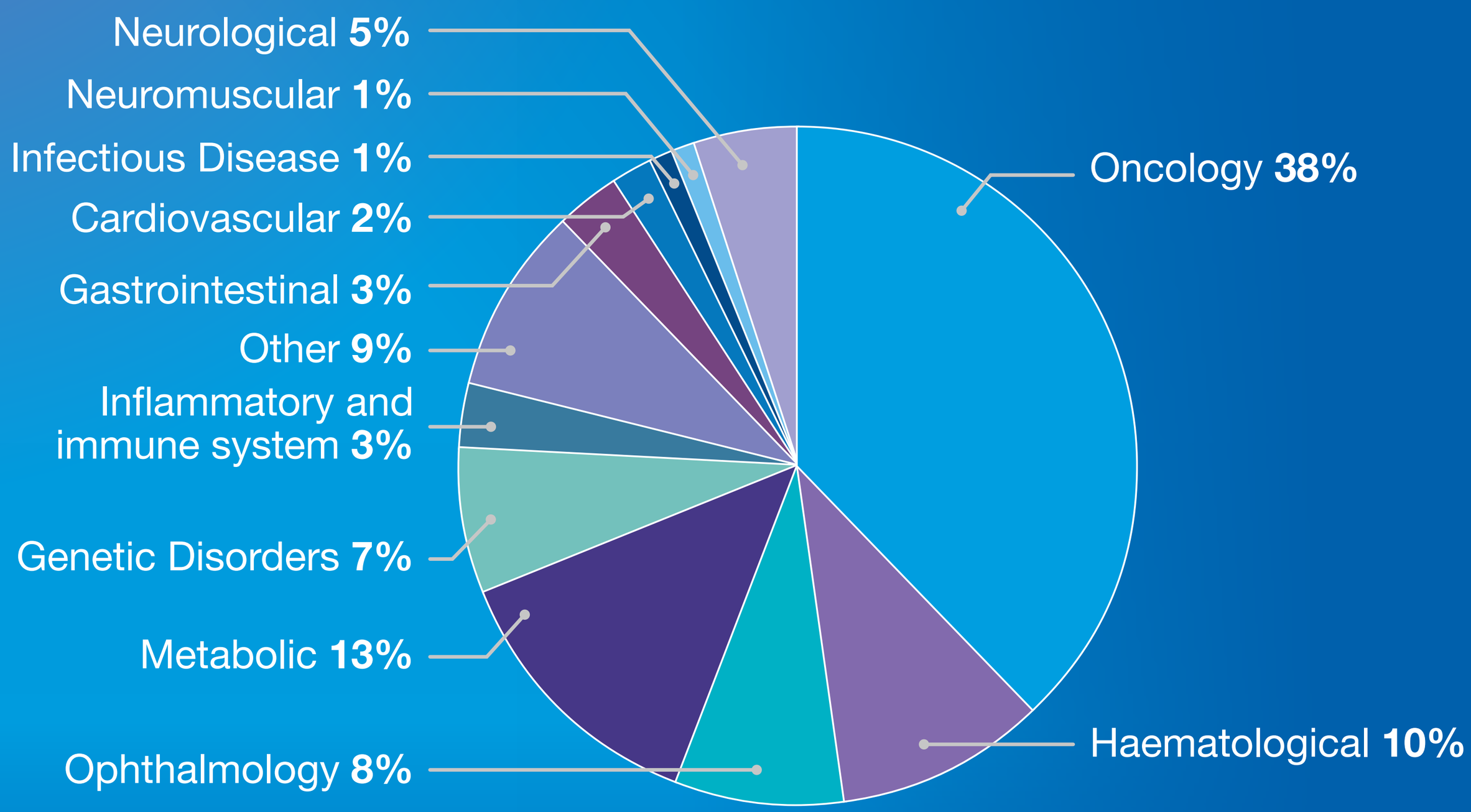
With an even distribution between *in vivo* and *ex vivo* gene therapy trials.



Percentages have been rounded to the nearest whole number.

Oncology remains the dominant therapeutic area under investigation

Therapies looking into metabolic indications have grown noticeably from last year (from 8% to 13%).



Percentages have been rounded to the nearest whole number.

Read on for the full 2024 Cell and Gene Therapy Catapult ATMP Clinical Trials Database

Cell and Gene Therapy Catapult ATMP Clinical Trials Database 2024

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)
AAVantgarde Bio Srl	A Phase I/II and First-in-human Study to Assess Effectiveness and Safety Profile of AAVB-081 for the Treatment of MYOA7-related Usher Syndrome (USH1B)	NCT06591793 081-101	Phase I/II	Recruiting	Ophthalmology	Retinitis Pigmentosa	N/A	Yes, *in vivo*	AAV	N/A	2024	Moorfields Eye Hospital NHS Foundation Trust, London The Retina Clinic, London
AbbVie Inc	Pivotal 2 Study of RGX-314 Gene Therapy in Participants With nAMD (ASCENT)	NCT05407636 RGX-314-3101 jRCT2073240017	Phase III	Recruiting	Ophthalmology	Neovascular (wet) age-related macular degeneration	N/A	Yes, *in vivo*	AAV	N/A	2021	Liverpool University Hospitals NHS Foundation Trust, Liverpool Moorfields Eye Hospital NHS Foundation Trust, London The Retina Clinic, London Oxford University Hospitals, Oxford
Achilles Therapeutics UK Limited	ATL001 in Patients with Metastatic or Recurrent Melanoma	NCT03997474 ATX-ME-001 2018-003446-16 EudraCT-2024-513062-19 2024-513062-19-00 EUCT-2024-513062-19-00	Phase I/II	Terminated	Oncology (Other)	Metastatic or Recurrent Melanoma	T cells	No	N/A	Autologous	2019	The Christie NHS Foundation Trust, Manchester Freeman Hospital, Newcastle Hospitals NHS Foundation Trust, Newcastle Upon Tyne Cambridge University Hospitals NHS Foundation Trust, Cambridge University College London Hospitals NHS Foundation Trust, London Guy's and St Thomas' NHS Foundation Trust, London The Royal Marsden NHS Foundation Trust, London University Hospital Southampton NHS Foundation Trust, Southampton
Achilles Therapeutics UK Limited	ATL001 in Patients with Advanced Unresectable or Metastatic NSCLC	NCT04032847 ATX-NS-001 EudraCT-2024-513060-26	Phase I/II	Terminated	Oncology (Other)	Non-Small Cell Lung Cancer	T cells	No	N/A	Autologous	2019	University Hospitals Birmingham NHS Foundation Trust, Birmingham Cambridge University Hospitals NHS Foundation Trust, Cambridge The Christie NHS Foundation Trust, Manchester University College London Hospitals NHS Foundation Trust, London University Hospital Southampton NHS Foundation Trust, Southampton Freeman Hospital, Newcastle Hospitals NHS Foundation Trust, Newcastle Upon Tyne The Leeds Teaching Hospitals NHS Trust, Leeds Guy's and St Thomas' NHS Foundation Trust, London
Adaptimmune Therapeutics Plc	AFPC332T in Advanced HCC	ADP-0033-001 GDCT0254617 GDC30009397 NCI-2017-01188 CDR789757 16111GCC UMGCC16111GCC 20160692 XX-0056 201903120	Phase I	In follow-up	Oncology (Other)	Hepatocellular Carcinoma Liver Cancer	T cells	Yes, *ex vivo*	Lentivirus	Autologous	2017	Beatson West of Scotland Cancer Centre, Glasgow Guy's and St Thomas' NHS Foundation Trust, London University College London Hospital Clinical Research Facility, London The Christie NHS Foundation Trust, Manchester
Adaptimmune Therapeutics Plc	Spearhead 1 Study in Subjects with Advanced Synovial Sarcoma or Myxoid/Round Cell Liposarcoma	NCT04044768 ADP 0044-002 NCI-2019-05482 2019-000589-39 EudraCT-2019-000589-39 20251 19194 OSU-19130 ADP-0044-002 201904212 RG1005467 19-004764 17621 CRUK-17621	Phase II	Recruiting	Oncology (Other)	Synovial Sarcoma,Myxoid/ Round Cell Liposarcoma	T cells	Yes, *ex vivo*	Lentivirus	Autologous	2019	University College London Hospital Clinical Research Facility, London The Christie NHS Foundation Trust, Manchester
Adaptimmune Therapeutics Plc	ADP-A2M4CD8 Monotherapy and in Combination with Nivolumab in HLA-A2+ Subjects with MAGE-A4 Positive Ovarian Cancer (SURPASS-3)	GOG-3084 NCT05601752 ADP-0055-003 EudraCT-2022-003176-16 2022-003176-16 ADP-0055-003/GOG-3084	Phase II	Recruiting	Oncology (Other)	Head And Neck Cancer Squamous Cell CarcinomaOral Cavity (Mouth) CancerOropharyngeal CancerHypopharyngeal CancerLaryngeal CancerRecurrent Head And Neck Cancer Squamous Cell Carcinoma	T cells	Yes, *ex vivo*	Lentivirus	Autologous	2023	University College London Hospitals NHS Foundation Trust, London The Royal Marsden NHS Foundation Trust, London The Christie NHS Foundation Trust, Manchester

Cell and Gene Therapy Catapult ATMP Clinical Trials Database 2024

Sponsor	Title	Clinical Database Numbers	Trial Phase	Trial Status	Disease Area	Indication	Cell Type	Gene Modification / Gene Therapy	Type of Vector Used (if applicable)	Autologous/ Allogeneic	Year Trial Started	United Kingdom Site(s)
Adaptimmune Therapeutics Plc	Master Protocol to Assess the Safety and Antitumor Activity of Genetically Engineered T Cells in NY-ESO-1 and/?or LAGE-1a Positive Solid Tumors (IGNYTE-ESO)	NCT03967223 208467 2019-000415-87 EudraCT-2019-000415-87 NCI-2019-05288 XX-0091 19292 OSU-19245 201907126 NL-OMON52674 NL70428.000.19 EUCTR2019-000415-87-NL 2024-513032-14-00 EUCT-2024-513032-14-00 CTIS2024-513032-14-00	Phase II	In follow-up	Oncology (Other)	Synovial Sarcoma Solid Tumor	T cells	Yes, *ex vivo*	Lentivirus	Autologous	2019	University College London Hospitals NHS Foundation Trust, London The Royal Marsden NHS Foundation Trust, London The Christie NHS Foundation Trust, Manchester
Adverum Biotechnologies Inc	Safety and Efficacy of ADVM-022 in Treatment-Experienced Patients With Neovascular Age-related Macular Degeneration [LUNA]	NCT05536973 ADVM-022-11 ISRCTN42621450 IRAS 1006271 2022-002261-15 CPMS 53941	Phase II	In follow-up	Ophthalmology	Age-related Macular Degeneration (AMD)	N/A	Yes, *in vivo*	AAV	N/A	2022	Moorfields Eye Hospital NHS Foundation Trust, London John Radcliffe Hospital, Oxford
Akamis Bio Ltd	A Study of NG-641 and Pembrolizumab in Squamous Cell Carcinoma of the Head and Neck (MOAT)	NG-641-02	Phase I	In follow-up	Oncology (Other)	Squamous Cell Carcinoma of the Head and Neck	N/A	Yes, *in vivo*	Oncolytic Virus	N/A	2021	University Hospital of Wales, Cardiff The Clatterbridge Cancer Centre NHS Foundation Trust, Liverpool The Royal Marsden NHS Foundation Trust, London University Hospital Southampton NHS Foundation Trust, Southampton
Akamis Bio Ltd	Study of NG-641 in Combination with Nivolumab in Metastatic or Advanced Epithelial Tumours (NEBULA)	NG-641-03	Phase I	In follow-up	Oncology (Other)	Metastatic CancerEpithelial TumorTransitional Cell Carcinoma (Urothelial Cell Carcinoma) Non-Small Cell Lung CancerEndometrial CancerCervical CancerEsophageal CancerGastric CancerTriple-Negative Breast Cancer (TNBC)Hepatocellular CarcinomaSquamous Cell CarcinomaUterine CancerRecurrent Head And Neck Cancer Squamous Cell Carcinoma	N/A	Yes, *in vivo*	Adenovirus	N/A	2021	Oxford University Hospital, Oxford The Clatterbridge Cancer Centre NHS Foundation Trust, Liverpool
Akamis Bio Ltd	Study of NG-350A Plus Pembrolizumab in Metastatic or Advanced Epithelial Tumours (FORTIFY) (FORTIFY)	1005052 2022-000335-22 CPMS 52187 EudraCT-2022-000335-22 IRAS 1005052 ISRCTN94964671 KEYNOTE-C84 MK-3475-C84 NG-350A-02	Phase I	In follow-up	Oncology (Other)	Multiple Myeloma (Kahler Disease)	N/A	Yes, *in vivo*	Adenovirus	N/A	2022	Churchill Hospital, Oxford The Clatterbridge Cancer Centre NHS Foundation Trust, Liverpool
Akouos Inc	Gene Therapy Trial for Otoferlin Gene-mediated Hearing Loss (AK-OTOF-101)	NCT05821959 2023-505128-76-00	Phase I/II	Recruiting	Ear, nose, and throat	Otoferlin Gene (OTOF)-Mediated Hearing Loss	N/A	Yes, *in vivo*	AAV	N/A	2023	University College London Hospitals NHS Foundation Trust, London
AlloVir Inc	Study to Evaluate Viralym-M (ALVR105) for the Treatment of Virus-Associated Hemorrhagic Cystitis (HC)	NCT04390113 AVM-003-HC NCI-2020-04433 2020-000722-26 EudraCT-2020-000722-26 202100534 GDCT0410260 GDC30026557	Phase III	Terminated	Renal and Urogenital	Hemorrhagic Cystitis	T cells	No	N/A	Allogeneic	2021	Queen Elizabeth Hospital, Birmingham Nottingham University Hospitals NHS Trust, Nottingham University Hospitals Bristol and Weston NHS Foundation Trust, Bristol Great Ormond Street Hospital for hildren NHS Trust, London Hammersmith Hospital, London The Royal Marsden NHS Foundation Trust, London University College London Hospitals NHS Foundation Trust, London

Cell and Gene Therapy Catapult ATMP Clinical Trials Database 2024

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	Posoleucel (ALVR105) for the Treatment of Adenovirus Infection in Pediatric and Adult Participants Receiving Standard of Care Following Allogeneic Hematopoietic Cell Transplantation	NCT05179057 P-105-303 2021-003450-22 EudraCT-2021-003450-22	Phase III	Terminated	Infectious Disease	Adenoviridae Infections	T cells	No	N/A	Allogeneic	2022	Sheffield Children's NHS Foundation Trust, Sheffield Royal Manchester Children's Hospital, Manchester Bristol Royal Hospital for Children, Bristol Royal Hospital for Children, Glasgow Birmingham Childrens Hospital NHS Foundation Trust, Birmingham University College London Hospitals NHS Foundation Trust, London St Mary's Hospital, London Great Ormond Street Hospital for Children NHS Foundation Trust, London
AlloVir Inc	Study of Posoleucel (ALVR105, Viralym-M) for Multi-Virus Prevention in Patients Post-Allogeneic Hematopoietic Cell Transplant (Prevent)	202200117 NCT05305040 P-105-202 Phase 3	Phase II/III	Terminated	Infectious Disease	Treatment for 6 Viral Pathogens Potentially Transmitted Through Transplantation	T cells	No	N/A	Allogeneic	2022	Bristol Haematology and Oncology Centre, Bristol University Hospitals Bristol, Bristol Addenbrookes Hospital, Cambridge Queen Elizabeth University Hospital, Glasgow Great Ormond Street Hospital for Children NHS Foundation Trust, London
Amgen Inc	Postmarketing Prospective Study of Melanoma Patients Treated with Imlygic to Characterize Risk of Herpetic Infection	20130193 EUPAS15128 NCI-2019-06388	Phase IV	Recruiting	Oncology (Other)	Melanoma		Yes, *in vivo*	Oncolytic Virus	N/A	2017	Cheltenham General Hospital, Cheltenham Guy's and St Thomas' NHS Foundation Trust, London The Royal Marsden NHS Foundation Trust, London Churchill Hospital, Oxford Musgrove Park Hospital, Taunton
Amgen Inc	Talimogene Laherparepvec with Pembrolizumab in Melanoma Following Progression on Prior Anti-PD-1 Based Therapy (MASTERKEY-115)	20180115 2019-001906-61 NCI-2019-07131 EudraCT-2019-001906-61 2019-001906-61 (EudraCT Number)	Phase II	Completed	Oncology (Other)	Melanoma	N/A	Yes, *in vivo*	Oncolytic Virus	N/A	2020	Guy's and St Thomas' NHS Foundation Trust, London The Royal Marsden NHS Foundation Trust, London
Astellas Gene Therapies	Gene Transfer Study in Patients with Late Onset Pompe Disease (FORTIS)	AT845-01 2019-003595-38 EudraCT-2019-003595-38	Phase I/II	Recruiting	Metabolic (Other)	Late Onset Pompe Disease	N/A	Yes, *in vivo*	AAV	N/A	2021	The Newcastle upon Tyne Hospitals NHS Foundation Trust Clinical Trial Facility, Newcastle Upon Tyne
Astellas Pharma Inc	A Safety Surveillance Study in Subjects with Macular Degenerative Disease Treated with Human Embryonic Stem Cell-derived Retinal Pigment Epithelial Cell Therapy	NCT03167203 7316-CL-0007 RPE-SSS-001 2016-005245-23 EudraCT-2016-005245-23	Phase I/II	Recruiting	Ophthalmology	Age-related Macular Degeneration (AMD)	Retinal	No	N/A	Allogeneic	2018	Moorfields Eye Hospital NHS Foundation Trust, London
Atamyo Therapeutics SAS	GNT0006 Gene Therapy Trial in Patients with Limb-girdle Muscular Dystrophy	2021-004276-33 ATA-001-FKRP NCT05224505 GDC30029488 GDCT0441697 2021-004276-33 (EudraCT Number)	Phase I/II	Recruiting	Genetic Disorders	FKRP-related Limb-girdle Muscular Dystrophy (LGMDR9, Formerly LGMD2I)	N/A	Yes, *in vivo*	AAV	N/A	2022	University College London Hospitals NHS Foundation Trust, London

Cell and Gene Therapy Catapult ATMP Clinical Trials Database 2024

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	Tabelecleucel for Solid Organ or Allogeneic Hematopoietic Cell Transplant Participants With Epstein-Barr Virus-Associated Post-Transplant Lymphoproliferative Disease (EBV+ PTLD) After Failure of Rituximab or Rituximab and Chemotherapy (ALLELE)	1708018515 20005 20161025 201612068 2017-002949-30 2017-0769 2017-0771 ATA129-EBV-301 ATA129-EBV-302 EudraCT-2017-002949-30 GDC20006702 GDCT0253980 NCI-2018-00043 NCI-2018-00293 NCT03392142 NCT03394365 OSU-18035	Phase III	In follow-up	Oncology (Other)	Epstein-Barr Virus-Associated Post-Transplant Lymphoproliferative Disease	T cells	No	N/A	Allogeneic	2017	University Hospitals Birmingham NHS Foundation Trust, Birmingham Imperial College Healthcare NHS Trust, London King's College Hospital NHS Foundation Trust, London
Atara Biotherapeutics Inc	A Study to Evaluate Tabelecleucel in Participants with Epstein-barr Virus-associated Diseases	NCT04554914 ATA129-EBV-205 2020-000177-25 EudraCT-2020-000177-25 NCI-2020-07698 2020-000177-25 (EudraCT Number) 2020-000177-25 (EudraCT Number)	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 Hospitals Birmingham NHS Foundation Trust, Birmingham
Autolus Therapeutics Plc	Phase I/II Study Evaluating AUTO4 in Patients with TRBC1 Positive T Cell Lymphoma	NCT03590574 2017-001965-26 EudraCT-2017-001965-26 UK-0237 AUTO4-TL1	Phase I/II	In follow-up	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	University College London Hospitals NHS Foundation Trust, London Manchester Royal Infirmary, Manchester Queen Elizabeth University Hospital, Glasgow Freeman Hospital, Newcastle Hospitals NHS Foundation Trust, Newcastle Upon Tyne
Autolus Therapeutics Plc	Long-term Follow-up of Patients Treated with Genetically Modified Autologous t Cells	AUTO-LT1 2016-004867-38 EudraCT-2016-004867-38	Phase II	Recruiting	Oncology (Other)	Multiple Myeloma	T cells	Yes, *ex vivo*	Retrovirus	Autologous	2018	Manchester Royal Infirmary, Manchester Queen Elizabeth University Hospital, Glasgow University College London Hospitals NHS Foundation Trust, London Royal Manchester Children's Hospital, Manchester Freeman Hospital, Newcastle Hospitals NHS Foundation Trust, Newcastle Upon Tyne
Autolus Therapeutics Plc	A Study of CD19 Targeted CAR T Cell Therapy in Adult Patients with Relapsed or Refractory B Cell Acute Lymphoblastic Leukaemia (ALL)	2019-001937-16 NCT04404660 NCI-2020-04370 202004253 FELIX (AUTO1-AL1) 17562 CRUK-17562 GDC30020798 GDCT0356178 2019-001937-16 (EudraCT Number)	Phase I/II	In follow-up	Oncology (Haematology)	B-Cell Acute Lymphocytic Leukemia	T cells	Yes, *ex vivo*	Lentivirus	Autologous	2020	University Hospitals Bristol and Weston NHS Foundation Trust, Bristol Cambridge University Hospitals NHS Foundation Trust, Cambridge University College London Hospitals NHS Foundation Trust, London King's College Hospital NHS Foundation Trust, London Manchester Royal Infirmary Hospital, Manchester Queen Elizabeth University Hospital, Glasgow Freeman Hospital, Newcastle Hospitals NHS Foundation Trust, Newcastle Upon Tyne University Hospitals Birmingham NHS Foundation Trust, Birmingham
Autolus Therapeutics Plc	A Study of CD19 Targeted Car T Cell Therapy in Patients with Severe, Refractory Systemic Lupus Erythematosus (SLE) (CARLYSE)	NCT06333483 AUTO1-SL1 2023-508236-60-00 2023-508236-60-00 (EU Trial (CTIS) Number) EUCT-2023-508236-60-00	Phase I	Recruiting	Inflammatory and immune system	Systemic Lupus Erythematosus	T cells	Yes, *ex vivo*	Lentivirus	Autologous	2024	University College London Hospitals NHS Foundation Trust, London Manchester Royal Infirmary, Manchester
Autolus Therapeutics Plc	A Study of CD19 Targeted CAR T Cell Therapy in Pediatric Patients with Relapsed or Refractory B Cell Acute Lymphoblastic Leukaemia (B ALL) and Aggressive Mature B-cell Non-hodgkin Lymphoma (B NHL)	AUTO1-PY1 2023-506307-26-00 2023-506307-26-00 (Other Identifier: EU CT)	Phase I	Recruiting	Oncology (Haematology)	B-Cell non-Hodgkin lymphoma	T cells	Yes, *ex vivo*	Lentivirus	Autologous	2023	Great Ormond Street Hospital for Children NHS Foundation Trust, London Great North Childrens Hospital, Newcastle Upon Tyne

Cell and Gene Therapy Catapult ATMP Clinical Trials Database 2024

Sponsor	Title	Clinical Database Numbers	Trial Phase	Trial Status	Disease Area	Indication	Cell Type	Gene Modification / Gene Therapy	Type of Vector Used (if applicable)	Autologous/ Allogeneic	Year Trial Started	United Kingdom Site(s)
Autolus Therapeutics Plc	Multi-modular Chimeric Antigen Receptor Targeting GD2 in Neuroblastoma (MAGNETO)	NCT05990751 UCL\142424	Phase I	Recruiting	Oncology (Other)	Neuroblastoma	T cells	Yes, *ex vivo*	Retrovirus	Autologous	2024	Great Ormond Street Hospital for Children NHS Foundation Trust, London
Barts Health NHS Trust	Use of Bone Marrow Derived Stem Cell and G-CSF with Circulatory Assistance in the Treatment of DCM (DCM-Support)	Reda 012357 2018-001063-23 EudraCT-2018-001063-23 2018-001063-23 (EudraCT Number)	Phase II	In follow-up	Cardiovascular	Dilated Cardiomyopathy	Bone marrow mononuclear cells	No	N/A	Autologous	2018	St Bartholomew's Hospital, London
Bayer AG	Study to Test the Safety and How Well Patients with Severe Hemophilia a Respond to Treatment with Bay 2599023 (Dtx 201), a Drug Therapy That Delivers a Healthy Version of the Defective Factor Viii Gene into the Nucleus of Liver Cells Using an Altered, Non-infectious Virus (Aav) as a "Shuttle"	19429 BAY19429 NCT03588299 2017-000806-39 EudraCT-2017-000806-39 1708-1646 US-1646 2023-505827-29-00 BAY2599023/19429 NL-OMON55861 NL66590.000.18 EUCTR2017-000806-39-NL CTIS2023-505827-29-00 2017-000806-39 (EudraCT Number)	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
Beacon Therapeutics (USA) Inc	A Clinical Trial Evaluating the Safety and Efficacy of a Single Subretinal Injection of AGTC-501 in Participants With XLRP	NCT04850118 AGTC-RPGR-002 EUCT-2024-511181-36-00	Phase II/III	Recruiting	Ophthalmology	X-linked Retinitis Pigmentosa	N/A	Yes, *in vivo*	AAV	N/A	2024	Moorfields Eye Hospital NHS Foundation Trust, London The Retina Clinic, London Oxford Eye Hospital, Oxford
Beam Therapeutics Inc	A Study to Evaluate the Safety and Efficacy of BEAM-302 in Adult Patients With Alpha-1 Antitrypsin Deficiency (AATD)	BTX-302-001 NCT06389877	Phase I/II	Recruiting	Genetic Disorders	Alpha-1 Antitrypsin Deficiency	N/A	Yes, *in vivo*	Non-viral	N/A	2024	Clinical Study Centre, London
Belfast Health and Social Care Trust	Repair of Acute Respiratory Distress Syndrome by Stromal Cell Administration (REALIST)(REALIST)	16154DMcA-AS NCT03042143 EudraCT-2017-000584-33 2017-000584-33 227090 40531	Phase I/II	In follow-up	Respiratory	Acute Respiratory Distress Syndrome (ARDS) due to COVID-19 or other causes of ARDS	Mesenchymal stem/stromal cells	No	N/A	Allogeneic	2019	Belfast Health and Social Care Trust, Belfast
Bellicum Pharmaceuticals Inc	Safety Study of Gene Modified Donor T-cells Following TCRaß+ Depleted Stem Cell Transplant	EudraCT-2014-000584-41 2014-000584-41 BP-004 IRAS-170965	Phase I/II	Completed	Oncology (Haematology)	Haematological Malignancies	T cells	Yes, *ex vivo*	Retrovirus	Allogeneic	2020	Great Ormond Street Hospital for Children NHS Foundation Trust, London Great North Children's Hospital, Newcastle Upon Tyne Royal Free Hospital, London Freeman Hospital, Newcastle Hospitals NHS Foundation Trust, Newcastle Upon Tyne

Cell and Gene Therapy Catapult ATMP Clinical Trials Database 2024

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)
Biogen Inc	Long-term Safety and Efficacy Follow-up of BIIB111 for the Treatment of Choroideremia and BIIB112 for the Treatment of X-Linked Retinitis Pigmentosa (SOLSTICE)	NCT03584165	Phase III	Recruiting	Ophthalmology	Choroideremia	N/A	Yes, *in vivo*	AAV	N/A	2018	Moorfields Eye Hospital NHS Foundation Trust, London Manchester Royal Eye Hospital, Manchester John Radcliffe Hospital, Oxford Southampton General Hospital, Southampton
BioMarin Pharmaceutical Inc	Gene Therapy Study in Severe Haemophilia A Patients (270-201)	270-201 GDC40001726 GDCT0211369 2014-003880-38 BMN270-201 NCT02576795 UKCRN-19239 HAEM 4448 IRAS 173490 UKCTG-5877 BMN 270-201	Phase I/II	Completed	Haematological	Hemophilia A (Factor VIII Deficiency)	N/A	Yes, *in vivo*	AAV	N/A	2015	The Royal London Hospital, London Queen Elizabeth Hospital, Birmingham Guy's and St Thomas' NHS Foundation Trust, London Addenbrooke's Hospital, Cambridge University Hospital Southampton NHS Foundation Trust, Southampton
BioMarin Pharmaceutical Inc	Single-Arm Study To Evaluate The Efficacy and Safety of Valoctocogene Roxaparvovec in Hemophilia A Patients (BMN 270-301) (BMN 270-301)	Study 270-301 NCT03370913 2017-003215-19 EudraCT-2017-003215-19 XX-0071 270-301	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 Bart's and The London School of Medicine and Dentistry, London Hammersmith Hospital, London Guy's and St Thomas' NHS Foundation Trust, London Churchill Hospital, Oxford University Hospital Southampton NHS Foundation Trust, Southampton Royal Cornwall Hospital, Cornwall
BioMarin Pharmaceutical Inc	Gene Therapy Study in Severe Haemophilia A Patients with Antibodies Against AAV5(GENEr8-AAV5+)	NCT03520712 BMN 270-203 EudraCT-2017-000662-29 2017-000662-29 UK-0233 202200029 GDCT0447396 Study 270-203 2017-000662-29 (EudraCT Number)	Phase I/II	Terminated	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	AAV Gene Therapy Study for Subjects With PKU	Study-201 2019-001878-28 EudraCT-2019-001878-28 307-201 BMN 307-201 NCT04480567	Phase I/II	In follow-up	Metabolic (Other)	Phenylketonuria (PKU)	N/A	Yes, *in vivo*	AAV	N/A	2020	University Hospitals Birmingham NHS Foundation Trust, Birmingham
BioMarin Pharmaceutical Inc	A Long-Term Follow-Up Study in Severe Hemophilia A Subjects Who Received BMN 270 in a Prior BioMarin Clinical Trial (270-401) (GENEr8-LTE)	BMN 270-401 2022-001246-38	Phase IV	Recruiting	Haematological	Heamophilia A	N/A	Yes, *in vivo*	AAV	N/A	2023	Addenbrookes Hospital, Cambridge Hammersmith Hospital, London Queen Elizabeth Hospital, Birmingham Royal London Hospital, London Guy's and St Thomas' NHS Foundation Trust, London University Hospital Southampton NHS Foundation Trust, Southampton Churchill Hospital, Oxford
BioNTech SE	Safety and Preliminary Efficacy Trial of BNT142 in Patients With CLDN6 Positive Solid Tumors	NCT05262530 BNT142-01 2021-005481-18 EudraCT-2021-005481-18 NL-OMON53910 NL83219.000.23 EUCTR2021-005481-18-NL CTIS2024-512639-58-00 4781/0029 BNT142-01 (4781/0029)	Phase I/II	Recruiting	Oncology (Other)	Advanced Solid Tumors	N/A	Yes, *in vivo*	N/A	N/A	2022	Cambridge University Hospitals NHS Foundation Trust, Cambridge The Clatterbridge Cancer Centre NHS Foundation Trust, Liverpool Guy's and St Thomas' NHS Foundation Trust, London Sarah Cannon Research Institute, London Nottingham University Hospitals NHS Trust, Nottingham Churchill Hospital, Oxford

Cell and Gene Therapy Catapult ATMP Clinical Trials Database 2024

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)
Bluebird bio Inc	Long-term Follow-up of Participants with Cerebral Adrenoleukodystrophy who were treated with Lenti-D Drug Product	NCT02698579 LTF-304 IRAS-129575 UKCRN-20304 CHIL 5109 2015-002805-13 NCI-2019-08553 RBR-72wbbg U1111-1249-1366 06580819.1.0000.0068 3.163.804 3,163,804 STUDY00005330 2015-002805-13 (EudraCT Number) CTIS2024-513904-33-00 EUCTR2015-002805-13-NL NL76175.000.21 NL-OMON54368 2024-513904-33-00 EUCT-2024-513904-33-00	Phase III	In follow-up	Metabolic (Other)	Adrenoleukodystrophy	CD34 and/or CD133 stem cells	Yes, *ex vivo*	Lentivirus	Autologous	2016	Great Ormond Street Hospital for Children NHS Foundation Trust, London Royal Free London Hospital, London
Bluebird bio Inc	Long-term Follow-up of Subjects With Transfusion-Dependent ß-Thalassemia (TDT) Treated With *ex vivo* Gene Therapy	LTF-303 2013-002245-11 EudraCT-2013-002245-11 NCI-2020-03443 2024-513905-29-00	Phase III	In follow-up	Haematological	Sickle Cell Disease β-Thalassemia	CD34 and/or CD133 stem cells	Yes, *ex vivo*	Lentivirus	Autologous	2014	University College London Hospitals NHS Foundation Trust, London
Boston Children's Hospital	Phase I/II Trial of Lentiviral Gene Transfer for SCID-X1 with Low Dose Targeted Busulfan Conditioning	P00023098 US-1737	Phase I/II	In follow-up	Inflammatory and immune system	X-Linked SCIDSevere Combined Immune Deficiency	CD34 and/or CD133 stem cells	Yes, *ex vivo*	Lentivirus	Autologous	2018	Great Ormond Street Hospital for Children NHS Foundation Trust, London
Cancer Research UK	A Trial of ChAdOx1 and MVA Vaccines Against MAGE-A3 and NY-ESO-1	CRUKD/20/001 2019-003015-64 17221 CUKD/20/001 2019-003015-64 (EudraCT Number) EudraCT-2019-003015-64	Phase I/II	Suspended	Oncology (Other)	Non-Small Cell Lung Cancer and Squamous Oesophageal Cancer	N/A	Yes, *in vivo*	Adenovirus	N/A	2021	The Christie NHS Foundation Trust, Manchester St James' University Hospital, Leeds Southampton General Hospital, Southampton The Beatson West of Scotland Cancer Centre, Glasgow Churchill Hospital, Oxford Guy's and St Thomas' NHS Foundation Trust, London Leicester Royal Infirmary, Leicester Blackpool Victoria Hospital, Blackpool Ninewells Hospital, Dundee Royal Preston Hospital, Preston
Cancer Vaccines Ltd	Investigation of a Therapeutic Vaccine (ACIT-1) in Cancer	2012-005426-30 EudraCT-2012-005426-30 NCT03096093 ACIT-1-1001 IRAS28730	Phase I/II	In follow-up	Oncology (Other)	Pancreatic cancer	Antigen presenting cells	No	N/A	Allogeneic	2017	Liverpool University Hospitals NHS Foundation Trust, Liverpool The Clatterbridge Cancer Centre NHS Foundation Trust, Liverpool
Celgene Corp	Long-Term Follow-up Protocol for Subjects Treated With Gene-Modified T Cells	GC-LTFU-001 U1111-1206-8250 2017-001465-24 EudraCT-2017-001465-24 NCI-2018-00957 18082 study GC-LTFU-001	Phase II/III	Recruiting	Oncology (Other)	Neoplasms	T cells	Yes, *ex vivo*	Lentivirus	Autologous	2018	University College London Cancer Institute, London King's College Hospital NHS Foundation Trust, London The Christie NHS Foundation Trust, Manchester Somers Cancer Research Building, Southampton

Cell and Gene Therapy Catapult ATMP Clinical Trials Database 2024

Sponsor	Title	Clinical Database Numbers	Trial Phase	Trial Status	Disease Area	Indication	Cell Type	Gene Modification / Gene Therapy	Type of Vector Used (if applicable)	Autologous/ Allogeneic	Year Trial Started	United Kingdom Site(s)
Celgene Corp	Efficacy and Safety Study of Bb2121 Versus Standard Regimens in Subjects with Relapsed and Refractory Multiple Myeloma (RRMM) (KarMMa-3)	NCT03651128 BB2121-MM-003 U1111-1217-9988 2018-001023-38 EudraCT-2018-001023-38 NCI-2018-02057 US-1806 1808-1806 JapicCTI-194719 GDCT0344524 GDC20023220 PRO00100712 201808202 RECF3952 1905020064 MM-003 SNCTP000004086 BASEC2020-00777 jRCT1080224647 EUCTR2018-001023-38 2018-001023-38-FR 2018-001023-38 (EudraCT Number) bb2121-MM-003	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	An Efficacy and Safety Study of bb2121 in Subjects with Relapsed and Refractory Multiple Myeloma and in Subjects with High-Risk Multiple Myeloma (KarMMa-2)	BB2121-MM-002 U1111-1216-4209 2018-000264-28 EudraCT-2018-000264-28 NCI-2018-01730 MM-002 1807-1785 US-1785 GDCT0344500 GDC20023219 RECF3933 XX-0072 201809141	Phase II	Recruiting	Oncology (Haematology)	Multiple Myeloma	T cells	Yes, *ex vivo*	Lentivirus	Autologous	2018	King's College Hospital NHS Foundation Trust, London
Celgene Corp	A Study to Evaluate the Efficacy and Safety of Jcar017 in Adult Subjects with Relapsed or Refractory Indolent B-cell Non-hodgkin Lymphoma (NHL) (Transcend FL)	2024-510966-18 19-3011 2019-004081-18 21-013 EudraCT-2019-004081-18 HSR200067 JapicCTI-205250 JCAR017-FOL-001 jRCT1080225154 NCI-2020-02084 RG1007248 STU00212069 U1111-1244-9768 EUCT-2024-510966-18 2024-510966-18 (Other Identifier) (OTHER: EU CTR)	Phase II	In follow-up	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	University College London Hospitals NHS Foundation Trust, London The Christie NHS Foundation Trust, Manchester
Celgene Corp	A Study to Compare the Efficacy and Safety of Idecabtagene Vicleucel With Lenalidomide Maintenance Therapy Versus Lenalidomide Maintenance Therapy Alone in Adult Participants With Newly Diagnosed Multiple Myeloma Who Have Suboptimal Response After Autologous Stem Cell Transplantation (KarMMa-9)	NCT06045806 GDCT0498724 CA089-1043 2022-501346-30 U1111-1280-9736 jRCT2061230084 202400148 NCT06399393 EudraCT-2022-501346-30 2022-501346-30 (EudraCT Number)	Phase III	In follow-up	Oncology (Other)	Multiple Myeloma (Kahler Disease)	T cells	Yes, *ex vivo*	Lentivirus	Autologous	2023	Queen Elizabeth Hospital, Birmingham Gartnavel General Hospital, Glasgow Southampton General Hospital, Southampton King's College Hospital NHS Foundation Trust, London Hammersmith Hospital, London

Cell and Gene Therapy Catapult ATMP Clinical Trials Database 2024

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	EXCELLENT (EXpanded CELL ENdocardiac Transplantation) (EXCELLENT)	NCT02669810 EudraCT 2014-001476-63 2014-001476-63 IRAS-157632 15/SS/0106 VHP590 (VHP2014137) VHP677 (VHP2015065)	Phase I/II	Completed	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 St Bartholomew's Hospital, London Ninewells Hospital and Medical School, Dundee University of Edinburgh, Edinburgh Queen Elizabeth Hospital, Birmingham
CellProthera SAS	A Phase III Clinical Study of ProtheraCytes in Post-myocardial Infarction	GDC30034137	Phase III	In planning/ set-up	Cardiovascular	Myocardial Infarction Congestive Heart Failure (Heart Failure)	CD34 and/or CD133 stem cells	No	N/A	Autologous	2024	UK sites not yet confirmed
CellProthera SAS	A Multicentre Observational Study to Assess Long-term Outcome of Participants in the EXCELLENT Clinical Trial (PERFECT)	CPT-MI-R01	Observational	In planning/ set-up	Cardiovascular	Myocardial Infarction	CD34 and/or CD133 stem cells	No	N/A	Autologous	2024	Queen Elizabeth Hospital, Birmingham University of Edinburgh, Edinburgh St. Bartholomew's Hospital, London Ninewells Hospital & Medical School, Dundee
Cook MyoSite Inc	Autologous Muscle Derived Cells Compared to Placebo for Urinary Sphincter Repair in Post-surgical Female Stress Incontinence (CELLEBRATE)	15-06 2017-000956-25 EudraCT-2017-000956-25	Phase III	Recruiting	Renal and Urogenital	Stress Urinary Incontinence	Skeletal Muscle	No	N/A	Autologous	2019	University Hospital Plymouth NHS Trust, Plymouth
CSL Ltd	HOPE-B: Trial of AMT-061 in Severe or Moderately Severe Haemophilia B Patients	NCT03569891 2017-004305-40 EudraCT-2017-004305-40 00113507 B/BE/18/BVW7 1803-1742 US-1742 XX-0089 CSL222_3001 (CT-AMT-061-02) CT-AMT-061-02 IND 016248 CSL222-3001 NL65714.000.18 EUCTR2017-004305-40-NL CTIS2024-510738-42-00 NL-OMON52495 AMT6102 2024-510738-42-00 EUCT-2024-510738-42-00 AMT6102 HOPE-B	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
Encoded Therapeutics Inc	A Clinical Study to Evaluate the Safety and Efficacy of ETX101, an AAV9-Delivered Gene Therapy in Children With SCN1A-positive Dravet Syndrome (UK Only)	NCT06283212 ETX-DS-005	Phase I/II	Recruiting	Neurological	Dravet Syndrome	N/A	Yes, *in vivo*	AAV	N/A	2024	Queen Elizabeth Hospital, Glasgow Great Ormond Street Hospital for Children NHS Foundation Trust, London Sheffield Children's Hospital, Sheffield
Ethris GmbH	A Phase I, First in Human Clinical Study of ETH-47 for Treatment and Prophylaxis of Respiratory Viral Infections in Healthy Subjects	ISRCTN15391202 ETH47-101 IRAS 1007882 1007882	Phase I	In follow-up	Respiratory	Viral Respiratory Tract Infection	N/A	Yes, *in vivo*	Non-viral	N/A	2023	Simbec Research Ltd, Merthyr Tydfil

Cell and Gene Therapy Catapult ATMP Clinical Trials Database 2024

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)
Gamida Cell Ltd	Stem Cell Transplantation With NiCord (Omidubicel) vs Standard UCB in Patients With Leukemia, Lymphoma, and MDS	NCT02730299 GC P05.01.020 2016-000704-28 EudraCT-2016-000704-28 GCP 05.01.020 NCI-2016-01518 CDR784490 1870GCCC UMGCC1870GCCC DUKE-00072499 STUDY00018301 STUDY00018301 17-252 MOH_2018-08-27_002477 GC P#05.01.020 P0501	Phase III	In follow-up	Oncology (Haematology)	Leukemia, Lymphoma, and Myelodysplastic Syndrome (MDS)	CD34 and/or CD133 stem cells	No	N/A	Allogeneic	2016	The Royal Marsden NHS Foundation Trust, London Queen Elizabeth Hospital, Birmingham St James Hospital, Leeds
Genethon SA	Microdystrophin (Gnt0004) Gene Therapy Clinical Trial in Duchenne Muscular Dystrophy a Phase I/Ii/Iii Study with a Dose Determination Part Followed by an Efficacy and Safety Evaluation, Quadruple Blind Placebo-controlled Part and Then by a Long Term Safety Follow up Part, in Ambulant Boys	2020-002093-27 EudraCT-2020-002093-27 GNT-016-MDYF	Phase I	Recruiting	Neuromuscular	Duchenne Muscular Dystrophy	N/A	Yes, *in vivo*	AAV	N/A	2021	Great Ormond Street Hospital for Children NHS Foundation Trust, London
Genethon SA	Gene Therapy for X-linked Chronic Granulomatous Disease (X-CGD) (CGD)	G1XCGD.01 2012-000242-35 UKCRN-13647 MREC-N-GTAC196 EudraCT-2012-000242-35 SNCTP000001301 EUCTR2012-000242-35	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 Foundation Trust, London University College London Hospitals NHS Foundation Trust, London
Genethon SA	Long Term Safety Follow up of Haematopoietic Stem Cell Gene Therapy for the Wiskott Aldrich Syndrome (WASFUP)	GNT-WAS-03 NCT02333760 UKCRN17206 17206 148367 FR-0063 IRAS ID 148367 2014-000274-20 2024-512680-31-00 EUCT-2024-512680-31-00	Phase I/II	In follow-up	Inflammatory and immune system	Wiskott-Aldrich Syndrome	CD34 and/or CD133 stem cells	Yes, *ex vivo*	Lentivirus	Autologous	2014	University College London Great Ormond Street Institute of Child Health, London
GenSight Biologics SA	Efficacy & Safety Study of Bilateral IVT Injection of GS010 in LHON Subjects Due to the ND4 Mutation for up to 1 Year (REFLECT)	GS-LHON-CLIN-05 2017-002187-40 EudraCT-2017-002187-40 1803-1728 US-1728	Phase III	Completed	Ophthalmology	Leber Hereditary Optic Neuropathy	N/A	Yes, *in vivo*	AAV	N/A	2019	Moorfields Eye Hospital NHS Foundation Trust, London
GenSight Biologics SA	Dose-escalation Study to Evaluate the Safety and Tolerability of GS030 in Subjects with Retinitis Pigmentosa (PIONEER)	NCT03326336 GS030_CLIN_001 2017-002204-27 EudraCT-2017-002204-27 2017-002204-27 (EudraCT Number)	Phase I/II	Recruiting	Ophthalmology	Retinitis Pigmentosa	N/A	Yes, *in vivo*	AAV	N/A	2018	Moorfields Eye Hospital NHS Foundation Trust, London
Gilead Sciences Inc	A Study to Compare Axicabtagene Ciloleucel With Standard of Care Therapy as First-line Treatment in Participants With High-risk Large B-cell Lymphoma(ZUMA-23)	KT-US-484-0136 2022-501489-24-00 GDCT0454791 ZUMA-23	Phase III	Recruiting	Oncology (Other)	Follicular Lymphoma, Diffuse Large B-Cell Lymphoma, Marginal Zone B-cell Lymphoma	T cells	Yes, *ex vivo*	Retrovirus	Autologous	2023	Addenbrookes Hospital, Cambridge University Hospital Southampton NHS Foundation Trust, Southampton

Cell and Gene Therapy Catapult ATMP Clinical Trials Database 2024

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)
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)	KT-EU-472-6036	Phase IV	Recruiting	Oncology (Haematology)	Mantle Cell Lymphoma	T cells	Yes, *ex vivo*	Retrovirus	Autologous	2022	UK sites not yet confirmed
Gilead Sciences Inc	Study of KITE-363 or KITE-753 in Participants With Relapsed and/or Refractory B-cell Lymphoma	NCT04989803 ; KT-US-499-0150; 2020-000562-41; EudraCT-2020-000562-41; NL-OMON53464; CTIS2024-511616-24-00; EUCTR2020-000562-41-NL; NL81647.000.22;	Phase I	Recruiting	Oncology (Other)	Relapsed/Refractory Follicular Lymphoma	T cells	Yes, *ex vivo*	Retrovirus	Autologous	2021	King's College Hospital NHS Foundation Trust, London
Gilead Sciences Inc	Long-term Follow-up Study for Participants of Kite-sponsored Interventional Studies Treated with Gene-modified Cells	KT-US-982-5968 2020-005843-21 EudraCT-2020-005843-21 IND 27453 27453 MOH_2022-08-21_010606 2023-507041-28 jRCT2013230062 EUCTR2020-005843-21 NL79971.000.22 EUCTR2020-005843-21-NL CTIS2023-507041-28-00 NL-OMON51477	Phase II	Recruiting	Oncology (Haematology)	Refractory Large B-Cell Lymphoma	T cells	Yes, *ex vivo*	Retrovirus	Autologous	2021	The Royal Marsden NHS Foundation Trust, London
Great Ormond Street Hospital for Children NHS Foundation Trust	TT52CAR19 Therapy for B-Cell Acute Lymphoblastic Leukaemia (B-ALL) (PBLTT52CAR19)	18IC07 2019-003462-40 NCT04557436	Phase I	In follow-up	Oncology (Haematology)	B-Cell Acute Lymphocytic Leukemia (B-Cell Acute Lymphoblastic Leukaemia)	T cells	Yes, *ex vivo*	Lentivirus	Allogeneic	2020	Great Ormond Street Hospital for Children NHS Foundation Trust, London
Great Ormond Street Hospital for Children NHS Foundation Trust	Base Edited CAR7 T Cells to Treat T Cell Malignancies (TvT CAR7) (TvT CAR7)	2021-004312-25 1004379 IRAS 1004379 19IC17 NCT05397184 EudraCT-2021-004312-25	Phase I	Recruiting	Oncology (Haematology)	T Cell Leukaemia	T cells	Yes, *ex vivo*	Non-viral	Allogeneic	2022	Great Ormond Street Hospital for Children NHS Foundation Trust, London
Great Ormond Street Hospital for Children NHS Foundation Trust	Lentiviral Gene Therapy for X-linked Severe Combined Immunodeficiency	16IC17 2018-000673-68 EudraCT-2018-000673-68 GDC30018638 GDCT0334500 UK-0236	Phase I/II	Recruiting	Inflammatory and immune system	X-Linked SCID Severe Combined Immune Deficiency (SCID)	CD34 and/or CD133 stem cells	Yes, *ex vivo*	Lentivirus	Autologous	2018	Great Ormond Street Hospital for Children NHS Foundation Trust, London
Great Ormond Street Hospital for Children NHS Foundation Trust	Long-term Follow-up after "Ready-made" CAR T Cells	1009055 IRAS-1009055 23IC21 IRAS 1009055	Phase IV	In planning/ set-up	Oncology (Haematology)	Acute Lymphocytic Leukemia (ALL)	T cells	Yes, *ex vivo*	Non-viral	Allogeneic	2024	UK sites not yet confirmed

Cell and Gene Therapy Catapult ATMP Clinical Trials Database 2024

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 Foundation Trust	Lentiviral Gene Therapy for p47 AR-CGD	19IC05	Phase I/II	Recruiting	Haematological	Chronic Granulomatous Disease	CD34 and/or CD133 stem cells	Yes, *ex vivo*	Lentivirus	Autologous	2023	Great Ormond Street Hospital for Children NHS Foundation Trust, London
Great Ormond Street Hospital for Children NHS Foundation Trust	Phase 1 study of base edited CAR T cells against AML: deep conditioning ahead of allogeneic stem cell transplantation	EudraCT Number: 2021-004311-66	Phase I	Recruiting	Oncology (Haematology)	Acute Myeloid Leukaemia (AML)	T cells	Yes, *ex vivo*	Lentivirus	Allogeneic	2023	Great Ormond Street Hospital for Children NHS Foundation Trust, London
Great Ormond Street Hospital for Children NHS Foundation Trust	Efficacy and safety of a cryopreserved formulation of autologous CD34+ hematopoietic stem cells transduced *ex vivo* with EFS lentiviral vector encoding for human ADA gene in subjects with severe combined immunodeficiency due to adenosine deaminase deficiency (OTL-101-5)	EudraCT No. 2017-001275-23	Phase I	In follow-up	Oncology (Haematology)	Severe Combined Immune Deficiency (SCID)	CD34 and/or CD133 stem cells	Yes, *ex vivo*	Lentivirus	Autologous	2018	Great Ormond Street Hospital for Children NHS Foundation Trust, London
GSK plc	Adoptive Cell Therapy Long-term Follow-up (LTFU) Study	ADP-0000-002 208750 17276 EudraCT-2018-004888-31 2018-004888-31 2018-0274 OSU-19040 20190078 NCI-2018-01288	Phase I	Recruiting	Oncology (Other)	Various	T cells	Yes, *ex vivo*	Lentivirus	Autologous	2018	University College London Hospitals NHS Foundation Trust, London The Christie NHS Foundation Trust, Manchester The Royal Marsden NHS Foundation Trust, London
GSK plc	Pilot Immunotherapy Study with Letetresgene Autoleucel (Lete-cel, GSK3377794)T-cells in New York Esophageal Squamous Cell Carcinoma-1 (NY-ESO-1)/ LAGE-1a-positive Advanced Non-small Cell Lung Cancer (NSCLC) Either Alone or in Combination with Pembrolizumab	208471 NCI-2018-03721 2018-003949-42 EudraCT-2018-003949-42 XX-0087 OSU-19071 201909026	Phase I/II	Terminated	Oncology (Other)	Non-Small Cell Lung Cancer	T cells	Yes, *ex vivo*	Lentivirus	Autologous	2019	GSK Investigational Site, London, The Christie NHS Foundation Trust, Manchester
GSK plc	Study to Evaluate Safety and Antitumor Activity of Lete-Cel (GSK3377794) in HLA-A2+ Participants With NY-ESO-1 Positive Previously Untreated Advanced (Metastatic or Unresectable) Synovial Sarcoma and Myxoid/Round Cell Liposarcoma	208467 Substudy 1	Phase II	In follow-up	Oncology (Other)	Advanced Synovial Sarcoma and Myxoid/ Round Cell Liposarcoma	T cells	Yes, *ex vivo*	Lentivirus	Autologous	2019	The Royal Marsden NHS Foundation Trust, London University College London Hospitals NHS Foundation Trust, London The Christie NHS Foundation Trust, Manchester
Guy's and St Thomas' NHS Foundation Trust King's College London	Treg Immunotherapy in Crohn's Disease (TRIBUTE)	2017-000170-11 EudraCT-2017-000170-11 NCT03185000	Phase I/II	In follow-up	Gastrointestinal (Other)	Crohn's Disease	T cells	No	N/A	Autologous	2022	Guy's and St Thomas' NHS Foundation Trust, London

Cell and Gene Therapy Catapult ATMP Clinical Trials Database 2024

Sponsor	Title	Clinical Database Numbers	Trial Phase	Trial Status	Disease Area	Indication	Cell Type	Gene Modification / Gene Therapy	Type of Vector Used (if applicable)	Autologous/ Allogeneic	Year Trial Started	United Kingdom Site(s)
Gyroscope Therapeutics Holdings Plc	Focus: A Phase I/II First in Human Study to Evaluate the Safety and Efficacy of GT005 Administered in Subjects with Dry AMD	GT005-01 2017-003712-39 EudraCT-2017-003712-39 CPPY988A12101	Phase I/II	Terminated	Ophthalmology	Age-related Macular Degeneration	N/A	Yes, *in vivo*	AAV	N/A	2018	Southampton General Hospital, Southampton London Vision Clinic, London Moorfields Eye Hospital NHS Foundation Trust, London Sunderland Eye Infirmary, Sunderland Manchester Eye Hospital, Manchester Bristol Eye Hospital, Bristol Oxford University Hospital, Oxford
Gyroscope Therapeutics Holdings Plc	EXPLORE: A Phase II Study to Evaluate the Safety and Efficacy of Two Doses of GT005 (EXPLORE)	GT005-02 2019-003421-22 NCT04437368 CPPY988A12202 NL71665.000.19 EUCTR2019-003421-22-NL	Phase II	Terminated	Ophthalmology	Age-related Macular Degeneration	N/A	Yes, *in vivo*	AAV	N/A	2020	Sunderland Eye Infirmary, Sunderland Bristol Eye Hospital, Bristol
Gyroscope Therapeutics Holdings Plc	HORIZON : A Phase II Study to Evaluate the Safety and Efficacy of Two Doses of GT005	NCT04566445 GT005-03 2020-002431-30 EudraCT-2020-002431-30 CPPY988A12201 2020-002431-30 (EudraCT Number)	Phase II	Terminated	Ophthalmology	Age-related Macular Degeneration	N/A	Yes, *in vivo*	AAV	N/A	2020	Sunderland Eye Infirmary, Sunderland Moorfields Eye Hospital NHS Foundation Trust, London John Radcliffe Hospital, Oxford London Vision Clinic, London
iECURE	An Open-label Study to Investigate ECUR-506 in Male Babies Less Than 9 Months of Age With Neonatal Onset OTC Deficiency (OTC-HOPE)	NCT06255782 ECUR-506-OTC-101 ISRCTN10957794 IRAS 1008261 1008261 CPMS 57410 2023-506180-34-01 EUCT-2023-506180-34-01	Phase I/II	In follow-up	Metabolic (Other)	Ornithine Transcarbanylase Deficiency	N/A	Yes, *in vivo*	AAV	N/A	2024	Great Ormond Street Hospital for Children NHS Foundation Trust, London Freeman Hospital, Newcastle Hospitals NHS Foundation Trust, Newcastle Upon Tyne
Inmune Bio Inc	A Phase I Open-label Dose Escalation Study of Intravenous Inkmune in Patients with MDS or AML (Laurel)	2019-004820-40 EudraCT-2019-004820-40 NCT05933070 INMB-INB16-002	Phase I	Terminated	Oncology (Other)	Myelodysplastic Syndrome	Other	No	N/A	Allogeneic	2020	Royal Hallamshire Hospital, Sheffield University Hospital Southampton NHS Foundation Trust, Southampton
Innovacell Biotechnologie AG	Skeletal Muscle-derived Cell Implantation for Treatment of Fecal Incontinence (Fidelia)	IC-01-02-5-009 2021-001376-42 EudraCT-2021-001376-42	Phase III	Recruiting	Gastrointestinal (Other)	Fecal Incontinence	Unknown	No	N/A	Autologous	2022	St Mary's Hospital, London
Instil Bio Inc	OVSTAR TIL Trial (OVarian Cancer Co-STimulatory Antigen Receptor TIL Trial) (OVSTAR)	NCT04389229 IMM-2019T-01-OC 2019-000106-30	Phase I/II	Withdrawn	Oncology (Other)	Metastatic Ovarian Cancer	T cells	Yes, *ex vivo*	Unknown	Autologous	2020	Queens Elizabeth Hospital, Birmingham The Christie NHS Foundation Trust, Manchester

Cell and Gene Therapy Catapult ATMP Clinical Trials Database 2024

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	Study to Evaluate Safety, Tolerability, Pharmacokinetics, and Pharmacodynamics of NTLA-2001 in Patients With Hereditary Transthyretin Amyloidosis With Polyneuropathy (ATTRv-PN) and Patients With Transthyretin Amyloidosis-Related Cardiomyopathy (ATTR-CM)	NCT04601051 ITL-2001-CL-001 2020-002034-32 EudraCT-2020-002034-32 2020-002034-32 (EudraCT Number)	Phase I	In follow-up	Metabolic (Other)	Hereditary Transthyretin Amyloidosis With Polyneuropathy (ATTRv-PN)	N/A	Yes, *in vivo*	Non-viral	N/A	2020	Clinical Trial Site, London
Intellia Therapeutics Inc	NTLA-2002 in Adults with Hereditary Angioedema (HAE) (NTLA-2002)	NCT05120830 ITL-2002-CL-001 2021-001693-33 EudraCT-2021-001693-33 NL-OMON54405 NL78549.000.21 EUCTR2021-001693-33-NL CTIS2024-512317-40-00	Phase I/II	In follow-up	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	Long-Term Follow-Up (LTFU) of Subjects Treated with NTLA 2002	ITL-2002-CL-999 2022-003778-22 2022-003778-22 (EudraCT Number) EudraCT-2022-003778-22	Phase I/II	Recruiting	Genetic Disorders	Hereditary Angioedema Deficiency)	N/A	Yes, *in vivo*	Non-viral	N/A	2024	Addenbrooke's Hospital, Cambridge
Intellia Therapeutics Inc	Magnitude: A Phase III Study of NTLA-2001 in Participants with Transthyretin Amyloidosis with Cardiomyopathy (ATTR-CM)	NCT06128629 ITL-2001-CL-301 EUCT-2023-507220-23-00 2023-507220-23-00 U1111-1291-3138 202400788	Phase III	Recruiting	Metabolic (Other)	Transthyretin Amyloidosis with Cardiomyopathy	N/A	Yes, *in vivo*	Non-viral	N/A	2023	Synexus Midlands Clinical Research Centre, Birmingham Synexus Wales Clinical Research Centre, Cardiff Synexus Scotland Clinical Research Centre, Glasgow Synexus Hexham Clinical Research Centre, Hexham Synexus Merseyside Clinical Research Centre. Liverpool Richmond Pharmacology, London Synexus Manchester Clinical Research Centre, Manchester
Iovance Biotherapeutics Inc	Study of Lifileucel (LN-144), Autologous Tumor Infiltrating Lymphocytes, in the Treatment of Patients with Metastatic Melanoma (LN-144)	C-144-01 GDC40003238 GDCT0231030 NCI-2015-01957 CDR769149 2017-000760-15 EudraCT-2017-000760-15 18309 P 54117 S16-00804 UKCTG-39529 2017-02031 BASEC2017-02031 EUCTR2017-000760-15 SNCTP000002819 2017-000760-15-GB	Phase II	In follow-up	Oncology (Other)	Metastatic Melanoma	T cells	No	N/A	Autologous	2015	The Royal Marsden NHS Foundation Trust, London Addenbrooke's Hospital, Cambridge Sarah Cannon Research Institute, London Beatson West of Scotland Cancer Centre, Glasgow
Iovance Biotherapeutics Inc	Study of LN-145, Autologous Tumor Infiltrating Lymphocytes in the Treatment of Patients With Cervical Carcinoma	NCT03108495 C-145-04 EudraCT-2016-003447-11 2016-003447-11 NCI-2017-01051 CDR789471 18959 P 38016 2017-0505 J17111 20161142 2017-010	Phase II	In follow-up	Oncology (Other)	Cervical Carcinomas	T cells	No	N/A	Autologous	2017	Bristol Haematology and Oncology Centre, Bristol Guy's and St Thomas' NHS Foundation Trust, London Sarah Cannon Research Institute, London University College London Hospitals NHS Foundation Trust, London NHS Greater Glasgow and Clyde, Glasgow

Cell and Gene Therapy Catapult ATMP Clinical Trials Database 2024

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)
Iovance Biotherapeutics Inc	Study of Autologous Tumor Infiltrating Lymphocytes in Patients with Solid Tumors	IOV-COM-202 2018-001608-12 EudraCT-2018-001608-12 NCI-2018-01950 EUCTR2018-001608-12 2018-001608-12-GB SNCTP000003376 BASEC2018-01915 COM-202 2018-01915 2018-001608-12 (EudraCT Number)	Phase II	Recruiting	Oncology (Other)	Metastatic Melanoma	T cells	No	N/A	Autologous	2019	Guy's and St Thomas' NHS Foundation Trust, London The Royal Marsden NHS Foundation Trust, London
Iovance Biotherapeutics Inc	Study to Investigate Lifileucel Regimen Plus Pembrolizumab Compared with Pembrolizumab Alone in Participants with Untreated Advanced Melanoma	NCT05727904 IOV-MEL-301 TILVANCE-301	Phase III	Recruiting	Oncology (Other)	Metastatic Melanoma	T cells	No	N/A	Autologous	2023	The Royal Marsden NHS Foundation Trust, London
Johnson & Johnson	Long-term Follow-up Gene Therapy Study for RPGR- XLRP	MGT010 2018-000425-31 NCT04312672 UK-0230	Phase I/II	In follow-up	Ophthalmology	X-Linked Retinitis Pigmentosa	N/A	Yes, *in vivo*	AAV	N/A	2017	Moorfields Eye Hospital NHS Foundation Trust, London Leeds Teaching Hospitals NHS Trust, Leeds
Johnson & Johnson	A Study Comparing JNJ-68284528, a CAR-T Therapy Directed Against B-cell Maturation Antigen (BCMA), Versus Pomalidomide, Bortezomib and Dexamethasone (PVd) or Daratumumab, Pomalidomide and Dexamethasone (DPd) in Participants with Relapsed and Lenalidomide-Refractory Multiple Myeloma (CARTITUDE-4)	CR108695 2019-001413-16 68284528MMY3002 EudraCT-2019-001413-16 NCI-2020-05552 OSU-20005 CT1175 MOH_2020-06-08_008780 MMY3002 jRCT1080225184 2019-001413-16 (EudraCT Number)	Phase III	In follow-up	Oncology (Haematology)	Multiple Myeloma	T cells	Yes, *ex vivo*	Lentivirus	Autologous	2020	Queen Elizabeth Hospital, Birmingham Bristol Haematology and Oncology Centre, Bristol University College London Hospitals NHS Foundation Trust, London King's College Hospital NHS Foundation Trust, London Freeman Hospital, Newcastle Hospitals NHS Foundation Trust, Newcastle Upon Tyne
Johnson & Johnson	Gene Therapy Trial for the Treatment of X-linked Retinitis Pigmentosa Associated With Variants in the RPGR Gene	2020-002873-88 EudraCT-2020-002873-88 IRAS-286986 EDMS-ERI-205374434 EUCTR2020-002873-88 CR109258 MGT-RPGR-021 2021-02177 SNCTP000004870 BASEC2021-02177 2020-002873-88-IE	Phase III	In follow-up	Ophthalmology	X-Linked Retinitis Pigmentosa	N/A	Yes, *in vivo*	AAV	N/A	2020	NHS Lothian, Edinburgh Gartnavel General Hospital, Glasgow Moorfields Eye Hospital NHS Foundation Trust, London St James's University Hospital, London
Johnson & Johnson	Follow-up Gene Therapy Trial for the Treatment of X-linked Retinitis Pigmentosa Associated with Variants in the RPGR Gene	MGT-RPGR-022 2020-002255-37 EudraCT-2020-002255-37 EUCTR2020-002255-37 2021-02178 SNCTP000004847 BASEC2021-02178 2020-002255-37-IE	Phase III	In follow-up	Ophthalmology	X-Linked Retinitis Pigmentosa	N/A	Yes, *in vivo*	AAV	N/A	2020	NHS Lothian, Edinburgh Gartnavel General Hospital, Glasgow St James University Hospital, Leeds Moorfields Eye Hospital NHS Foundation Trust, London

Cell and Gene Therapy Catapult ATMP Clinical Trials Database 2024

Sponsor	Title	Clinical Database Numbers	Trial Phase	Trial Status	Disease Area	Indication	Cell Type	Gene Modification / Gene Therapy	Type of Vector Used (if applicable)	Autologous/ Allogeneic	Year Trial Started	United Kingdom Site(s)
Johnson & Johnson	A Study of Bortezomib, Lenalidomide and Dexamethasone (VRd) Followed by Cilta-cel, a CAR-T Therapy Directed Against BCMA Versus VRd Followed by Lenalidomide and Dexamethasone (Rd) Therapy in Participants With Newly Diagnosed Multiple Myeloma for Whom ASCT is Not Planned as Initial Therapy (CARTITUDE-5)	CR109015 2021-001242-35 68284528MMY3004 jRCT2011210025 EudraCT-2021-001242-35 202100938 EUCTR2021-001242-35 BASEC2021-01822 SNCTP000005076 2021-001242-35-BE 2023-505850-16-00 2021-001242-35 (EudraCT Number) EUCT 2023-505850-16-00 2023-505850-16-00 (Registry Identifier) (REGISTRY: EUCT number) CTIS2023-505850-16-00	Phase III	In follow-up	Oncology (Haematology)	Multiple Myeloma	T cells	Yes, *ex vivo*	Lentivirus	Autologous	2021	University Hospitals Birmingham NHS Trust, Birmingham Bristol Royal Infirmary, Bristol Leeds Teaching Hospitals NHS Trust, Leeds University College London Hospitals NHS Foundation Trust, London King's College Hospital NHS Foundation Trust, London Manchester Royal Infirmary, Manchester The Royal Marsden NHS Foundation Trust, London
Johnson & Johnson	A Study to Evaluate Intravitreal JNJ-81201887 (AAVCAGsCD59) Compared to Sham Procedure for the Treatment of Geographic Atrophy (GA) Secondary to Age-related Macular Degeneration (AMD)	CR109236 81201887MDG2001 2022-500746-16-00 Eudra-CT 2022-500746-16-00 ISRCTN38641780 1006234 IRAS 1006234 CPMS 53494 BASEC2023-00555 EUCTR2022-500746-16 SNCTP000005519 2023-00555 EUCT- 2022-500746-16-00 CTIS2022-500746-16-00 2022-500746-16-00 (Registry Identifier) (REGISTRY: EUCT number)	Phase II	In follow-up	Ophthalmology	Dry (Atrophic) Macular Degeneration	N/A	Yes, *in vivo*	AAV	N/A	2023	Bristol Eye Hospital, Bristol Moorfields Eye Hospital NHS Foundation Trust, London Central Middlesex Hospital, London The Retina Clinic, London Manchester Royal Eye Hospital, Manchester Royal Victoria Infirmary, Newcastle upon Tyne Oxford Eye Hospital, Oxford University Hospital Southampton NHS Foundation Trust, Southampton
Johnson & Johnson	A Study of JNJ-90009530 in Relapsed or Refractory B-Cell Non-Hodgkin Lymphoma (r/r B-NHL)	90009530LYM1001 2023-506259-97-00 2023-506259-97 IRAS 1009316 1009316 GDC30033094 GDCT0486773 EudraCT-2023-506259-97 ISRCTN17666983 CPMS 57721 1009316	Phase I	Recruiting	Oncology	B-Cell non-Hodgkin Lymphoma	T cells	Yes, *ex vivo*	Lentivirus	Autologous	2023	University College London Hospitals NHS Foundation Trust, London
Johnson & Johnson	A Study of JNJ-90014496 in Participants With Relapsed or Refractory B-Cell Non-Hodgkin Lymphoma	NCT05421663 90014496LYM1001 2023-506267-33-00 2023-506267-33 1008775 IRAS 1008775 ISRCTN15038320 CPMS 58328 2023-506267-33 (EudraCT Number) 2023-506267-33-00 (Registry Identifier) (REGISTRY: EUCT number) EUCT-2023-506267-33-00 EudraCT-2023-506267-33 202400725	Phase I	Recruiting	Oncology	B-Cell non-Hodgkin Lymphoma	T cells	Yes, *ex vivo*	Lentivirus	Autologous	2022	University College London Hospitals NHS Foundation Trust, London The Christie NHS Foundation Trust, Manchester

Cell and Gene Therapy Catapult ATMP Clinical Trials Database 2024

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)
Juno Therapeutics Inc	A Study to Evaluate the Safety, Tolerability, Efficacy, and Drug Levels of CC-97540 in Participants With Relapsing Forms of Multiple Sclerosis or Progressive Forms of Multiple Sclerosis	NCT06220201 CA061-1006	Phase I	Recruiting	Neurological	Multiple Sclerosis	T cells	Yes, *ex vivo*	Lentivirus	N/A	2024	Royal London Hospital, London, Manchester Royal Infirmary, Manchester University College London Hospitals NHS Foundation Trust, London Salford Royal Hospital, Salford
King's College Hospital NHS Foundation Trust	Autologous Tregs for Aplastic Anaemia (TIARA)	2021-000082-33	Phase I	Recruiting	Haematological	Aplastic Anemia	T cells	No	N/A	Autologous	2022	King's College Hospital NHS Foundation Trust, London
King's College London	Phase I Trial: T4 Immunotherapy of Head and Neck Cancer	2012-001654-25 CDR748482 UKCRN-19183 ISRCTN81726461 19183 CRUK 12169 201-20016-54 12/LO/1834	Phase I	In follow-up	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	Study of Effectiveness of Axicabtagene Ciloleucel Compared to Standard of Care Therapy in Patients with Relapsed/ Refractory Diffuse Large B Cell Lymphoma (ZUMA-7)	NCT03391466 KTE-C19-107 2017-002261-22 EudraCT-2017-002261-22 016278 NCI-2018-00042 UMGCC1818GCCC 1818GCCC TEC-C19-107 OSU-17326 20729 2018-008 19341 SNCTP000003107 KTE-C19-107 (ZUMA-7) 2018-01359 MOH_2018-08-07_002200 17-541 2017-0699 113165 XX-0051 IND-016278 BASEC2018-01359 EUCTR2017-002261-22 2017-002261-22 (EudraCT Number) 2017-002261-22-GB KTE-C19-107	Phase III	In follow-up	Oncology (Haematology)	B-Cell Lymphoma	T cells	Yes, *ex vivo*	Retrovirus	Autologous	2018	University College London Hospitals NHS Foundation Trust, 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, London
Kite Pharma Inc	Study of Brexucabtagene Autoleucel (KTE-X19) in Participants With Relapsed/Refractory Mantle Cell Lymphoma	KTE-C19-102 (Cohort 3) 2015-005008-27 EudraCT- 2015-005008-27	Phase II	In follow-up	Oncology (Haematology)	Mantle Cell Lymphoma	T cells	Yes, *ex vivo*	Retrovirus	Autologous	2021	Queen Elizabeth University Hospital, Glasgow King's College Hospital NHS Foundation Trust, London Manchester Royal Infirmary, Manchester
Kite Pharma Inc	Study of Axicabtagene Ciloleucel versus Standard of Care Therapy in Participants with Relapsed/Refractory Follicular Lymphoma (ZUMA-22)	NCT05371093 KT-US-473-0133 2021-003260-28 EudraCT-2021-003260-28 jRCT2063230096 2024-511594-30-00 EUCT-2024-511594-30-00	Phase III	Recruiting	Oncology (Other)	Relapsed/ Refractory Follicular Lymphoma	T cells	Yes, *ex vivo*	Retrovirus	Autologous	2022	University Hospitals Birmingham NHS Foundation Trust, Birmingham Cambridge University Hospitals NHS Foundation Trust, Cambridge University College London Hospitals NHS Foundation Trust, London The Royal Marsden NHS Foundation Trust, London Kings College Hospital NHS Foundation Trust, London The Christie NHS Foundation Trust, Manchester Oxford University Hospitals, Oxford The Unversity Hospital Southampton NHS Foundation Trust, Southampton

Cell and Gene Therapy Catapult ATMP Clinical Trials Database 2024

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	A Study to Evaluate the Long-term Safety of Patients with Advanced Lymphoid Leukemia Who Have Been Previously Administered with UCART19	CL1-68587-003 2016-000297-38 EudraCT-2016-000297-38 NCI-2017-00302 CDR787274 17-425 200596 2016-0727	Phase I	In follow-up	Oncology (Haematology)	Advanced Lymphoid Malignancies	T cells	Yes, *ex vivo*	Lentivirus	Allogeneic	2021	King's College Hospital NHS Foundation Trust, London Great Ormond Street Hospital for Children NHS Foundation Trust, London
Leucid Bio Ltd	LEU01101: Safety and Preliminary Efficacy of LEU011 in Solid Tumours	NCT06193902 LEU01101	Phase I/II	Recruiting	Oncology (Other)	Relapsed/Refractory Solid Tumours	T cells	Yes, *ex vivo*	Retrovirus	Autologous	2023	Guy's and St Thomas' NHS Foundation Trust, London The Christie NHS Foundation Trust, Manchester
Mario Negri Institute for Pharmacological Research	NEPHSTROM for Diabetic Kidney Disease (NEPHSTROM)	634086 H2020-PHC-2014 PHC-13-2014 NUIG 089-15 EudraCT -2016-000661-23 GDCT0270832 GDC30011730 2016-000661-23 EUDRACT N°2016-000661-23	Phase I/II	Completed	Renal and Urogenital	Diabetic kidney disease (type 2 diabetes and chronic kidney disease)	Mesenchymal stem/stromal cells	No	N/A	Allogeneic	2017	Belfast City Hospital, Belfast University Hospitals Birmingham NHS Foundation Trust, Birmingham
MeiraGTx Holdings Plc	Long-term Follow-up Gene Therapy Study for Achromatopsia CNGB3 and CNGA3	MGT007 2016-003856-59 EudraCT-2016-003856-59 IRAS-216171 UK-0232	Phase I/II	Completed	Ophthalmology	Achromatopsia	N/A	Yes, *in vivo*	AAV	N/A	2017	Moorfields Eye Hospital NHS Foundation Trust, London
MeiraGTx Holdings Plc	A Study of AAV2-hAQP1 Gene Therapy in Participants With Radiation-Induced Late Xerostomia (AQUAX2)	NCT05926765 GDCT0491928 MGT-AQP1-201	Phase II	Recruiting	Oral	Xerostomia	N/A	Yes, *in vivo*	AAV	N/A	2023	Addenbrookes Hospital, Cambridge Ninewells Hospital and Medical School, Dundee Western General Hospital, Edinburgh Guy's and St Thomas' NHS Foundation Trust, London Nottingham University Hospitals NHS Foundation Trust, Nottingham
Mina Therapeutics Ltd	First-in-human Safety and Tolerability Study of MTL-CEBPA in Patients with Advanced Liver Cancer (OUTREACH)	MNA-3521-011 UKCRN-20332 CANC 4818 GDC20006440	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 Foundation Trust, Cambridge The Beatson West of Scotland Cancer Centre, Glasgow The Clatterbridge Cancer Centre NHS Foundation Trust, Liverpool Guy's and St Thomas' NHS Foundation Trust, London Imperial College Healthcare NHS Foundation Trust, London University College London Hospitals NHS Foundation Trust, London Freeman Hospital, Newcastle Hospitals NHS Foundation Trust, Newcastle Upon Tyne

Cell and Gene Therapy Catapult ATMP Clinical Trials Database 2024

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)
Moderna Inc	A Study to Assess Safety, Pharmacokinetics, and Pharmacodynamics of mRNA-3705 in Participants with Isolated Methylmalonic Acidemia	mRNA-3705-P101 2020-004980-24 EudraCT-2020-004980-24 2022-502492-32-00 EUCT-2022-502492-32-00 IND 28037 2020-004980-24 (EudraCT Number) 2020-004980-24-ES mRNA-3705-P101 EUCTR2020-004980-24 NL-OMON55911 CTIS2022-502492-32-00 EUCTR2020-004980-24-NL NL81760.000.22	Phase I/II	Recruiting	Metabolic (Other)	Isolated Methylmalonic Acidemia Due to Methylmalonyl-CoA Mutase Deficiency	N/A	Yes, *in vivo*	Non-viral	N/A	2021	Royal Manchester Children's Hospital, Manchester Birmingham Children's Hospital NHS Foundation Trust, Birmingham
Moderna Inc	Open-Label Study of mRNA-3927 in Participants with Propionic Acidemia	NCT04159103 mRNA-3927-P101 2019-003529-36 EudraCT-2019-003529-36 IRAS ID 1003761 2022-502910-10 jRCT2043240044	Phase I/II	Recruiting	Metabolic (Other)	Propionic Acidemia	N/A	Yes, *in vivo*	Non-viral	N/A	2021	University Hospitals Birmingham NHS Foundation Trust, Birmingham Great Ormond Street Hospital for Children NHS Foundation Trust, London Manchester University NHS Foundation Trust, Manchester
Moderna Inc	An Extension Study to Evaluate the Long-Term Safety and Clinical Activity of mRNA-3705 in Participants Previously Enrolled in Other Clinical Studies of mRNA-3705	1004273 2021-000446-17 2022-501997-20-00 EudraCT-2021-000446-17 mRNA-3705-P101-EXT	Phase I/II	Recruiting	Metabolic (Other)	Methylmalonic Acidemia	N/A	Yes, *in vivo*	Non-viral	N/A	2022	Birmingham Children's Hospital NHS Foundation Trust, Birmingham Royal Manchester Childrens Hospital, Manchester
Moderna Inc	A Study to Assess the Long-term Safety and Clinical Activity of mRNA-3927 in Participants Previously Enrolled in the Mrna-3927-P101 Study	2022-502911-12-00 mRNA-3927-P101-EXT EUCT-2022-502911-12-00 2022-502911-12-00 (Other Identifier) (OTHER: Clinical Trials Information System (CTIS))	Phase I/II	Recruiting	Metabolic (Other)	Propionic Acidemia	N/A	Yes, *in vivo*	Non-viral	N/A	2021	University Hospitals Birmingham NHS Foundation Trust, Birmingham Great Ormond Street Hospital for Children NHS Foundation Trust, London
Montpellier University Hospital	A Study Evaluating the Efficacy of a Single Injection Autologous Adipose Derived Mesenchymal Stromal Cells in Patients with Knee Osteoarthritis (ADIPOA-2)	UF 9494 GDC20005759 GDCT0245316 EudraCT-2015-002125-19 9494 2015-002125-19 IRAS-196188	Phase II	In follow-up	Musculoskeletal	Osteoarthritis (knee)	Mesenchymal stem/stromal cells [[OR]] Adipose-derived stem cells	No	N/A	Autologous	2016	Cambridge University Hospitals NHS Foundation Trust, Cambridge
Moorfields Eye Hospital NHS Foundation Trust	A Study of Implantation of Retinal Pigment Epithelium in Subjects with Acute Wet Age Related Macular Degeneration	B4711001 2011-005493-37 EudraCT-2011-005493-37 IRAS-96163	Phase I	Completed	Ophthalmology	Acute Wet Age Related Macular Degeneration	Retinal	No	N/A	Allogeneic	2021	Moorfields Eye Hospital NHS Foundation Trust, London
Neurogene Inc	Gene Therapy Study for Children With CLN5 Batten Disease (CLN5-200)	NCT05228145 CLN5-200	Phase I/II	In follow-up	Genetic Disorders	Batten Disease	N/A	Yes, *in vivo*	AAV	N/A	2022	Great Ormond Street Hospital for Children NHS Foundation Trust, London

Cell and Gene Therapy Catapult ATMP Clinical Trials Database 2024

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)
Neurogene Inc	A Novel, Regulated Gene Therapy (Ngn-401) Study for Female Children With Rett Syndrome	NCT05898620 RTT-200	Phase I/II	Recruiting	Neurological	Rett Syndrome	N/A	Yes, *in vivo*	AAV	N/A	2023	Royal Hospital for Children and Young People, Edinburgh Manchester University NHS Foundation Trust, Manchester
NHS Blood and Transplant	Recovery and Survival of Stem Cell Originated Red Cells	2017-002178-38 CPMS: 39643 IRAS: 229563	Phase I	Recruiting	Haematological	Blood Transfusions	CD34 and/or CD133 stem cells	No	N/A	Allogeneic	2021	NHS Blood and Transplant, Cambridge Donor Centre, Cambridge NIHR Cambridge Clinical Research Facility, Cambridge
NHS Lothian and the University of Edinburgh	Cell Therapy for Acute Liver Injury Trial	2022-002584-29 1005258 AC22087 IRAS 1005258 EudraCT-2022-002584-29	Phase I	Recruiting	Gastrointestinal (Liver)	Acute Liver Injury	Monocytes	No	N/A	Allogeneic	2022	Edinburgh Royal Infirmary, Edinburgh
Novartis AG	PHE885 CAR-T Therapy in Adult Participants with Relapsed and Refractory Multiple Myeloma	NCT05172596 CPHE885B12201 EudraCT-2021-003747-22 2021-003747-22 jRCT2013220012 202300592 2021-003747-22 (EudraCT Number)	Phase II	In follow-up	Oncology (Haematology)	Refractory Multiple Myeloma	T cells	Yes, *ex vivo*	Lentivirus	Autologous	2022	Novartis Investigative site, Birmingham Novartis Investigative site, Glasgow The Christie NHS Foundation Trust, Manchester
Novartis AG	CAR-T Long Term Follow Up (LTFU) Study (PAVO)	2014-001673-14 CDR774629 NCI-2015-01238 EudraCT-2014-001673-14 STU 032015-068 IRB00011960 OSU-15042 2015-0569 CTL019A2205B CCTL019A2205B JapicCTI-205095 2014-001673-14-AT CTR20220687 jRCT1080225004 2014-001673-14 (EudraCT Number) NL-OMON53025 CTL019A2205B (PAVO / CART-LTFU) CTIS2023-508128-37-00 EUCTR2014-001673-14-NL NL56825.000.16	Phase II/III	Recruiting	Oncology (Haematology)	Unspecified B-Cell Lymphomas B-Cell Leukemia	T cells	Yes, *ex vivo*	Lentivirus	Autologous	2016	Novartis Investigative Site, London
Novartis AG	Efficacy and Safety of Tisagenlecleucel in Adult Patients with Refractory or Relapsed Follicular Lymphoma (ELARA)	NCT03568461 CCTL019E2202 2017-004385-94 EudraCT-2017-004385-94 CT1030 NCI-2018-03720 JapicCTI-194610 18346 XX-0063 OSU-18248 217279 jRCT1080224541 NL66135.000.18 EUCTR2017-004385-94-NL CTIS2023-508127-13-00 NL-OMON55846	Phase II	In follow-up	Oncology (Haematology)	Follicular Lymphoma	T cells	Yes, *ex vivo*	Lentivirus	Autologous	2018	Queen Elizabeth Hospital, Birmingham King's College Hospital NHS Foundation Trust, London

Cell and Gene Therapy Catapult ATMP Clinical Trials Database 2024

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	Tisagenlecleucel in Adult Patients with Aggressive B-cell Non-hodgkin Lymphoma (BELINDA)	EudraCT-2016-002966-29 2016-002966-29 CCTL019H2301 NCT03570892 NCI-2018-02925 JapicCTI-194897 ES-0048 CTR20200561 OSU-18222 SNCTP000003400 BASEC2018-01892 IRB18-1014 STUDY00143907 jRCT1080224814 EUCTR2016-002966-29 2016-002966-29-DE 2016-002966-29 (EudraCT Number)	Phase III	In follow-up	Oncology (Haematology)	Non-Hodgkin Lymphoma	T cells	Yes, *ex vivo*	Lentivirus	Autologous	2019	Queen Elizabeth Hospital, Birmingham University College London Hospitals NHS Foundation Trust, London
Novartis AG	Study of Efficacy and Safety of Tisagenlecleucel in HR B-ALL EOC MRD Positive Patients (CASSIOPEIA)	CCTL019G2201J 2017-002116-14 NCT03876769 NCI-2019-02353 XX-0064 19376 PHO-NOVARTIS-CCTL019G2201J 201910182 AALL1721/CASSIOPEIA 507919 P/008/2019 P/215/2022 NL-OMON54738 CTIS2023-508081-15-00 EUCTR2017-002116-14-NL NL66137.000.18 2017-002116-14 (EudraCT Number) CTL019G2201J	Phase II	In follow-up	Oncology (Haematology)	ALL, B-cell Leukaemia	T cells	Yes, *ex vivo*	Lentivirus	Autologous	2019	University College London Hospitals NHS Foundation Trust, London Great Ormond Street Hospital for Children NHS Foundation Trust, London
Novartis AG	Long-term Follow-up of Patients With Spinal Muscular Atrophy Treated With OAV101 in Clinical Trials (SPECTRUM)	COAV101A12308 2021-006781-21 EudraCT-2021-006781-21 NMRR ID-23-03026-I7U jRCT2033230642 CTR20240883	Phase III	Recruiting	Neuromuscular	Spinal Muscular Atrophy	N/A	Yes, *in vivo*	AAV	N/A	2022	Royal Victoria Infirmary, Newcastle Upon Tyne Great Ormond Street Hospital for Children NHS Foundation Trust, London
Novartis Gene Therapies	Long-term Follow-up Study of Patients Receiving Onasemnogene Abeparvovec-xioi	AVXS-101-LT-002 EudraCT-2019-002611-26 2019-002611-26 JapicCTI-205305 LT-002 COAV101A12103 jRCT1080225209 2019-002611-26 (EudraCT Number) 205305	Phase III	In follow-up	Neuromuscular	Spinal Muscular Atrophy	N/A	Yes, *in vivo*	AAV	N/A	2020	Great Ormond Street Hospital for Children NHS Foundation Trust, London Freeman Hospital, Newcastle Hospitals NHS Foundation Trust, Newcastle Upon Tyne
Orchard Therapeutics Plc	A Study to Investigate the Efficacy and Safety of OTL-203 in Subjects with MPS-IH Compared with Standard of Care with Allogeneic Hsct (HURCULES)	NCT06149403 OTL-203-02	Phase III	Recruiting	Metabolic (Other)	Mucopolysaccharidosis I (MPS I) (Hurler Syndrome)	CD34 and/or CD133 stem cells	Yes, *ex vivo*	Lentivirus	Autologous	2023	Manchester University NHS Foundation Trust, Manchester

Cell and Gene Therapy Catapult ATMP Clinical Trials Database 2024

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)
Passage Bio Inc	Study of Safety, Tolerability and Efficacy of PBKR03 in Pediatric Subjects with Early Infantile Krabbe Disease (GALax-C)	NCT04771416 PBKR03-001 2020-005229-95 EudraCT-2020-005229-95	Phase I/II	Suspended	Neurological	Krabbe Disease	N/A	Yes, *in vivo*	AAV	N/A	2022	Manchester University NHS Foundation Trust, Manchester
Passage Bio Inc	Study of Safety, Tolerability and Efficacy of PBGM01 in Pediatric Participants with GM1 Gangliosidosis (Imagine-1)	EudraCT-2020-001109-22 2020-001109-22 PBGM01-001 NCT04713475	Phase I/II	In follow-up	Metabolic (Other)	GM1 Gangliosidosis	N/A	Yes, *in vivo*	AAV	N/A	2021	Great Ormond Street Hospital for Children NHS Foundation Trust, London
Pfizer Inc	A Study to Evaluate the Efficacy and Safety of Factor IX Gene Therapy with PF-06838435 in Adult Males with Moderately Severe to Severe Hemophilia B (BENEGENE-2)	C0371002 2018-003086-33 EudraCT-2018-003086-33 JapicCTI-205228 jRCT1080225132 EUCT-2022-502844-11-00 202300481 KCT0008736 2023-03-019 2022-502844-11-00 IND-016437 2018-003086-33 (EudraCT Number) 2022-502844-11-00 (EU CT Number)	Phase III	In follow-up	Haematological	Hemophilia B (Factor IX Deficiency)	N/A	Yes, *in vivo*	AAV	N/A	2019	Freeman Hospital, Newcastle Hospitals NHS Foundation Trust, Newcastle Upon Tyne Glasgow Royal Infirmary, Glasgow
Pfizer Inc	Study to Evaluate the Safety and Efficacy of PF-06939926 for the Treatment of Duchenne Muscular Dystrophy	NCT04281485 C3391003 2019-002921-31 EudraCT-2019-002921-31 MOH_2020-07-07_009124 KCT0005683 02-2020-027 2020-06-142 FP00018226 00127944 jRCT2051210011 SNCTP000004472 BASEC2020-02024 IRAS- 275551 EUCTR2019-002921-31 2019-002921-31-GB 2019-002921-31 (EudraCT Number) 2023-508510-42-00 EUCT-2023-508510-42-00 CTIS2023-508510-42-00	Phase III	In follow-up	Neuromuscular	Duchenne Muscular Dystrophy	N/A	Yes, *in vivo*	AAV	N/A	2020	Great Ormond Street Hospital for Children NHS Foundation Trust, London Royal Liverpool and Broadgreen University Hospitals NHS Trust, Liverpool Royal Victoria Infirmary, Newcastle upon Tyne Alder Hey Children's NHS Foundation Trust, Liverpool
Pfizer Inc	Phase III, Open-Label, Single-Arm Study to Evaluate the Efficacy and Safety of PF-07055480 (Recombinant AAV2/6 Human Factor VIII Gene Therapy) in Adult Male Participants with Moderately Severe to Severe Hemophilia A (FVIII:C=1%)	NCT04370054 C3731003 2019-004451-37 EudraCT-2019-004451-37 KCT0005828 202100117 jRCT2043210021 2019-004451-37 (EudraCT Number)	Phase III	In follow-up	Haematological	Haemophilia A	N/A	Yes, *in vivo*	AAV	N/A	2020	Guy's and St Thomas' NHS Foundation Trust, London
Prevail Therapeutics Inc	Phase I/II Clinical Trial of PR001 in Infants With Type 2 Gaucher Disease (PROVIDE)	NCT04411654 STUDY00008823 J3Z-MC-OJAB (PRV-GD2-101)	Phase I/II	In follow-up	Metabolic (Other)	Lysosomal Storage Disorder (Type 2 Gaucher disease)	N/A	Yes, *in vivo*	AAV	N/A	2021	Manchester Centre for Genomic Medicine, Manchester

Cell and Gene Therapy Catapult ATMP Clinical Trials Database 2024

Sponsor	Title	Clinical Database Numbers	Trial Phase	Trial Status	Disease Area	Indication	Cell Type	Gene Modification / Gene Therapy	Type of Vector Used (if applicable)	Autologous/ Allogeneic	Year Trial Started	United Kingdom Site(s)
Prevail Therapeutics Inc	Phase I/II Clinical Trial of LY3884963 in Patients with Frontotemporal Dementia with Progranulin Mutations (FTD-GRN)(PROCLAIM)	NCT04408625 EudraCT-2019-003159-12 2019-003159-12 019511 J4B-MC-OKAA (PRV-FTD101) US IND 019511	Phase I/II	Recruiting	Neurological	Frontotemporal Dementia	N/A	Yes, *in vivo*	AAV	N/A	2020	University College London Hospitals NHS Foundation Trust, London
Prevail Therapeutics Inc	A Clinical Trial of PR001 (LY3884961) in Patients with Peripheral Manifestations of Gaucher Disease (PROCEED)	J3Z-MC-OJAE	Phase I/II	Recruiting	Metabolic (Other)	Gaucher Disease	N/A	Yes, *in vivo*	AAV	N/A	2022	Royal Free Hospital, London
Prime Medicine Inc	A Clinical Study of PM359 for Chronic Granulomatous Disease	NCT06559176 Prime-0101	Phase I/II	In planning/ set-up	Genetic Disorders	Chronic Granulomatous Disease	CD34 and/or CD133 stem cells	Yes, *ex vivo*	Lentivirus	Autologous	2024	University College London Hospitals NHS Foundation Trust, London
Psivac Ltd	Ixovex-1 Single Agent and Combination Therapy (IXACT)	Ixovex-1-01	Phase I/II	In planning/ set-up	Oncology (Other)	Solid Tumor	N/A	Yes, *in vivo*	Adenovirus	N/A	2024	The Royal Marsden NHS Foundation Trust, London
Quell Therapeutics Ltd	Safety and Clinical Activity of QEL-001 in A2-mismatch Liver Transplant Patients (LIBERATE)	NCT05234190 QEL-001-CLN-01 2021-001379-18 EudraCT-2021-001379-18 2021-001379-18 (EudraCT Number) EUCT-2024-516193-30-01 2024-516193-30-01	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 Hospital, London King's College Hospital NHS Foundation Trust, London
Recode Therapeutics Inc	A Phase I Study Evaluating Safety and Tolerability of RCT2100 in Healthy Participants and in Participants with CF	2024-512169-15 RCT2100-101 2024-512169-15-00 EUCT-2024-512169-15-00 EudraCT-2024-512169-15 2024-512169-15 (EudraCT Number)	Phase I	Recruiting	Respiratory	Cystic Fibrosis	N/A	Yes, *in vivo*	Non-viral	N/A	2024	University Hospitals Birmingham NHS Foundation Trust, Birmingham Royal Papworth Hospital, Cambridge Leeds Teaching Hospitals, Leeds King's College Hospital NHS Foundation Trust, London Nottingham University Hospitals, Nottingham University Hospital Southampton NHS Foundation Trust, Southampton
Recode Therapeutics Inc	Study Evaluating the Safety and Tolerability of RCT1100 in Healthy and PCD Subjects	RCT1100-101	Phase I	In follow-up	Respiratory	Primary Ciliary Dyskinesia	N/A	Yes, *in vivo*	Non-viral	N/A	2023	Royal Brompton Hospital, London

Cell and Gene Therapy Catapult ATMP Clinical Trials Database 2024

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)
Recode Therapeutics Inc	A Study to Assess the Safety, Tolerability, Ciliary Rescue, and Pharmacodynamics of RCT1100 in Adults with Pcd	RCT1100-102	Phase I	Recruiting	Respiratory	Primary Ciliary Dyskinesia	N/A	Yes, *in vivo*	Non-viral	N/A	2024	Royal Brompton Hospital, London University Hospital Southampton NHS Foundation Trust, Southampton
Regeneron Pharmaceuticals Inc	A Study of DB-OTO, an Adeno-associated Virus (AAV) Based Gene Therapy, in Children/Infants With Hearing Loss Due to Otoferlin Mutations (CHORD)	NCT05788536 DB-OTO-001 2022-000079-38 EudraCT-2022-000079-38 2022-000079-38 (EudraCT Number) GDC30032310 GDCT0477424	Phase I/II	Recruiting	Ear, nose, and throat	Hearing Loss Due to Otoferlin Mutations (CHORD)	N/A	Yes, *in vivo*	AAV	N/A	2023	Cambridge University Hospitals NHS Foundation Trust, Cambridge Great Ormond Street Hospital for Children NHS Foundation Trust, London
RegenxBio Inc	A First-in-human Study in Pediatric Patients with Ocular Cln2 Disease	NCT05791864 RGX-381-1102 2021-000173-92	Phase I/II	In follow-up	Genetic Disorders	Batten Disease	N/A	Yes, *in vivo*	AAV	N/A	2023	Great Ormond Street Hospital for Children NHS Foundation Trust, London
Region Skane	A Trial to Determine the Safety and Tolerability of Transplanted Stem Cell Derived Dopamine Neurons to the Brains of Individuals With Parkinson's Disease (STEM-PD)	CCTU0263 2021-001366-38 EudraCT-2021-001366-38 GDC30033208 GDCT0489967 IRAS ID - 1005502	Phase I/II	In follow-up	Neurological	Parkinson's Disease	Other	No	N/A	Allogeneic	2022	Cambridge University Hospitals NHS Foundation Trust, Cambridge
Replimune Ltd	Study of RP1 Monotherapy and RP1 in Combination with Nivolumab (IGNYTE)	2016-004548-12 EudraCT-2016-004548-12 NCT03767348 RPL-001-16 20180802 S17-01386	Phase I/II	Recruiting	Oncology (Other)	Melanoma	N/A	Yes, *in vivo*	Oncolytic Virus	N/A	2017	The Clatterbridge Cancer Centre NHS Foundation Trust, Liverpool Southampton General Hospital, Southampton Oxford University Hospitals NHS Foundation Trust, Oxford University of Leeds Teaching Hospital, Leeds The Royal Marsden NHS Foundation Trust, London St James's University Hospital, Leeds Beatson West of Scotland Cancer Center, Glasgow
Replimune Ltd	Study of RP2 Monotherapy and RP2 in Combination with Nivolumab in Patients with Solid Tumors	NCT04336241 RP2-001-18 GDC30019213 GDCT0339757	Phase I	Recruiting	Oncology (Other)	Solid TumorTriple-Negative Breast Cancer	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
Replimune Ltd	Study of RP3 Monotherapy and RP3 in Combination with Nivolumab in Patients with Solid Tumours	RP3-301 NCT04735978	Phase I	In follow-up	Oncology (Other)	Advanced Solid Tumor	N/A	Yes, *in vivo*	Oncolytic Virus	N/A	2020	The Clatterbridge Cancer Centre NHS Foundation Trust, Liverpool Churchill Hospital, Oxford The Royal Marsden NHS Foundation Trust, London

Cell and Gene Therapy Catapult ATMP Clinical Trials Database 2024

Sponsor	Title	Clinical Database Numbers	Trial Phase	Trial Status	Disease Area	Indication	Cell Type	Gene Modification / Gene Therapy	Type of Vector Used (if applicable)	Autologous/ Allogeneic	Year Trial Started	United Kingdom Site(s)
Replimune Ltd	Study of Rp3 in Combination With Nivolumab and Other Therapy in Patients With Locoregionally Advanced or Recurrent Scchn	NCT05743270 RP3-002	Phase II	Withdrawn	Oncology (Other)	Head And Neck Cancer Squamous Cell Carcinoma Oral Cavity (Mouth) Cancer Oropharyngeal Cancer Hypopharyngeal Cancer Laryngeal Cancer Recurrent Head And Neck Cancer Squamous Cell Carcinoma	N/A	Yes, *in vivo*	Oncolytic Virus	N/A	2023	The Royal Marsden NHS Foundation Trust, London
Replimune Ltd	Study of RP2 in Combination With Second-line Therapy in Patients With Locally Advanced Unresectable or Metastatic HCC	NCT05733598 RP2-003	Phase II	Recruiting	Oncology (Other)	Hepatocellular CarcinomaMetastatic Hepatocellular Carcinoma	N/A	Yes, *in vivo*	Oncolytic Virus	N/A	2024	Churchill Hospital, Oxford The Royal Marsden NHS Foundation Trust, London Clatterbridge Cancer Centre NHS Foundation Trust, Clatterbridge The Royal Marsden NHS Foundation Trust, London
Resolution Therapeutics Ltd	An Open-label Phase I/II Multicentre Study to Evaluate the Safety, Tolerability and Efficacy of RTX001 Autologous Macrophages in Participants with Liver Cirrhosis who have Hepatic Decompensation	GDC30035065	Phase I/II	Recruiting	Gastrointestinal (Liver)	Decompensated Cirrhosis Liver Diseases	Monocytes	Yes, *ex vivo*	Non-viral	Autologous	2024	Royal Edinburgh Infirmary, Edinburgh
RHEACELL GmbH & Co KG	Allogeneic ABCB5-positive Dermal Mesenchymal Stromal Cells for Treatment of Therapy-resistant CVU (Phase III)	allo-APZ2-CVU-III 2024-512720-11-00 U1111-1305-5114 2024-512720-11-00 (EU Trial (CTIS) Number) NCT06489028	Phase III	Recruiting	Dermatological	Venous Leg Ulcers (Crural Ulcer)	Mesenchymal stem/stromal cells	No	N/A	Allogeneic	2024	RHEACELL Clinical Trial Sites, UK
Rocket Pharmaceuticals Inc	A Phase I/II Registrational Study of RPL-102 in Subjects with Fanconi Anemia	NCT04069533	Phase II	In follow-up	Genetic Disorders	Fanconi Anemia	CD34 and/or CD133 stem cells	Yes, *ex vivo*	Lentivirus	Autologous	2019	Great Ormond Street Hospital for Children NHS Foundation Trust, London
Rocket Pharmaceuticals Inc	Lentiviral-mediated Gene Therapy for Pediatric Patients With Fanconi Anemia Subtype A	RP-L102-0118 2018-002502-31 NCT04069533	Phase II	In follow-up	Haematological	Fanconi Anemia	CD34 and/or CD133 stem cells	Yes, *ex vivo*	Lentivirus	Autologous	2019	Great Ormond Street Hospital for Children NHS Foundation Trust, London
Rocket Pharmaceuticals Inc	Long-term Follow-up (LTFU) for Gene Therapy of Leukocyte Adhesion Deficiency-I (LAD-I)	RP-L201-0121-LTFU 2022-501086-41-00 EUCT-2022-501086-41-00	Phase I/II	Recruiting	Haematological	Leukocyte Adhesion Deficiency-I	CD34 and/or CD133 stem cells	Yes, *ex vivo*	Lentivirus	Autologous	2022	Great Ormond Street Hospital for Children NHS Foundation Trust, London

Cell and Gene Therapy Catapult ATMP Clinical Trials Database 2024

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)
Rocket Pharmaceuticals Inc	A Gene Therapy Study of RP-A501 in Male Patients with Danon Disease	NCT06092034 RP-A501-0123 GDCT0501290	Phase II	In follow-up	Metabolic (Other)	Danon Disease	N/A	Yes, *in vivo*	AAV	N/A	2023	Great Ormond Street Hospital for Children NHS Foundation Trust, London
Sangamo Therapeutics	Safety and Tolerability Study of Chimeric Antigen Receptor T-Reg Cell Therapy in Living Donor Renal Transplant Recipients (STEADFAST)	EudraCT-2019-001730-34 TX200-KT02 NCT04817774 EUCT-2024-512579-11-00	Phase I/II	In follow-up	Renal and Immune System	Kidney Transplant Rejection	T cells	Yes, *ex vivo*	Lentivirus	Autologous	2022	Oxford University Hospitals NHS Foundation Trust, Oxford
Sangamo Therapeutics	Long-Term Follow-Up of TX200-TR101 (STEADFAST)	EudraCT-2022-002440-40 TX200-KT03 NCT05987527 2022-002440-40 (EudraCT Number) 2024-512580-31-00 EUCT-2024-512580-31-00	Phase III	Recruiting	Renal and Immune System	Kidney Transplant Rejection	T cells	Yes, *ex vivo*	Lentivirus	Autologous	2023	Oxford University Hospitals NHS Foundation Trust, Oxford
Sangamo Therapeutics Inc	Dose-Ranging Study of ST-920, an AAV2/6 Human Alpha Galactosidase A Gene Therapy in Subjects With Fabry Disease (STAAR)	NCT04046224 ST-920-201 EudraCT-2019-000667-24	Phase I/II	In follow-up	Metabolic (Other)	Fabry Disease	N/A	Yes, *in vivo*	AAV	N/A	2019	Queen Elizabeth Hospital, Birmingham Addenbrooke's Hospital, Cambridge Royal Free Hospital, London
Sangamo Therapeutics Inc	Long-Term Follow-up of Subjects Who Were Treated with ST-920	ST-920-LT01 NCT05039866 EudraCT 2024-512700-18-00	Phase I/II	Recruiting	Metabolic (Other)	Fabry Disease	N/A	Yes, *in vivo*	AAV	N/A	2021	Addenbrooke's Hospital, Cambridge Royal Free Hospital, London
Sarepta Therapeutics Inc	A Gene Transfer Therapy Study to Evaluate the Safety and Efficacy of Delandistrogene Moxeparvovec (SRP-9001) in Participants with Duchenne Muscular Dystrophy (DMD) (EMBARK)	Study 301 SRP-9001-301 NCT05096221 2019-003374-91 jRCT2053210141 EudraCT-2019-003374-91 P/419/2020 9001-301	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 Royal Victoria Infirmary, Newcastle Upon Tyne
Sarepta Therapeutics Inc	A Long-term Follow-up Study of Participants Who Received Delandistrogene Moxeparvovec (SRP-9001) in a Previous Clinical Study (EXPEDITION)	SRP-9001-305 2023-505043-39-00 Study 9001-305 Study 305 jRCT2053240056 EUCT-2023-505043-39-00 U1111-1296-0489	Phase III	Recruiting	Genetic Disorders	Duchenne Muscular Dystrophy	N/A	Yes, *in vivo*	AAV	N/A	2023	Translational and Clinical Research Institute, Newcastle Upon Tyne

Cell and Gene Therapy Catapult ATMP Clinical Trials Database 2024

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)
Scancell Holdings Plc	A Phase II, Multicenter, Open-label Study of SCIB1 in Patients with Advanced Unresectable Melanoma Receiving Pembrolizumab	NCT04079166 GDCT0366648 SCIB1-002 2018-002844-10 EudraCT-2018-002844-10 IND 18331 CRUK 16281	Phase II	Recruiting	Oncology (Other)	Advanced Unresectable Melanoma	N/A	Yes, *in vivo*	Non-viral	N/A	2019	Cambridge University Hospitals NHS Foundation Trust, Cambridge Velindre University NHS Trust, Cardiff The Leeds Teaching Hospitals NHS Trust, Leeds Guy's and St. Thomas' NHS Foundation Trust, London The Royal Marsden NHS Foundation Trust, London The Christie NHS Foundation Trust, Manchester East and North Hertfordshire NHS Trust, Stevenage Nottingham University Hospitals NHS Trust; Nottingham Oxford University Hospital NHS Foundation Trust, Oxford University Hospital Plymouth NHS Trust, Plymouth Lancashire Teaching Hospitals NHS Foundation Trust, Preston Sheffield Teaching Hospitals NHS Trust, Sheffield University Hospital Southampton NHS Foundation Trust, Southampton Somerset NHS Foundation Trust, Taunton
Sensorion SA	A Phase I/II Clinical Trial with Sens-501 in Children Suffering from Severe to Profound Hearing Loss Due to Otoferlin (Otof) Mutations (AUDIOGENE)	NCT06370351 SENS-501-101 2023-504466-28-00 2023-504466-28-00 (EU Trial (CTIS) Number) EUCT-2023-504466-28-00	Phase I/II	Recruiting	Ear, nose, and throat	Deafness	N/A	Yes, *in vivo*	AAV	N/A	2024	UK sites not yet confirmed
Solid Biosciences Inc	Microdystrophin Gene Transfer Study in Adolescents and Children with DMD (IGNITE DMD)	NCT03368742 GX1001 EudraCT-2017-002213-60 2017-002213-60 US-1614	Phase I/II	In follow-up	Genetic Disorders	Duchenne Muscular Dystrophy	N/A	Yes, *in vivo*	AAV	N/A	2017	Newcastle upon Tyne Hospitals NHS Foundation Trust, Newcastle Upon Tyne
Spark Therapeutics Inc	A Gene Transfer Study for Late-Onset Pompe Disease (RESOLUTE)	NCT04093349 SPK-3006-101 2019-001283-30 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 Hospital, Salford Royal Free Hospital, London Queen Elizabeth Hospital, Birmingham
Spur Therapeutics Ltd	A Gene Therapy Study in Patients with Gaucher Disease Type 1 (GALILEO-1)	NCT05324943 FLT201-01 2020-005032-30 EudraCT-2020-005032-30	Phase I/II	In follow-up	Genetic Disorders	Gaucher Disease Type 1	N/A	Yes, *in vivo*	AAV	N/A	2022	Royal Free Hospital, London Salford Royal Hospital, Salford
Spur Therapeutics Ltd	Long Term Follow-up Study of Type-1 Gaucher Subjects Post FLT201 Dose (GALILEO-2)	2024-511172-33-00 FLT201-02 U1111-1303-0375 NCT06545136	Phase I/II	Recruiting	Metabolic (Other)	Gaucher Disease Type I	N/A	Yes, *in vivo*	AAV	N/A	2024	Salford Royal Hospital, Salford
St. Jude Children's Research Hospital Inc	Dose-escalation Study of A Self Complementary Adeno-associated Viral Vector For Gene Transfer in Hemophilia B	AGT4HB 5R01HL094396 EudraCT-2005-005711-17 2005-005711-17	Phase I	In follow-up	Haematological	Hemophilia B (Factor IX Deficiency)	N/A	Yes, *in vivo*	AAV	N/A	2010	University College London Hospitals NHS Foundation Trust, London

Cell and Gene Therapy Catapult ATMP Clinical Trials Database 2024

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)
Takeda Pharmaceutical Co Ltd	An Observational Post-marketing Registry on the Effectiveness and Safety of Darvadstrocel in Patients with Crohn's Disease and Complex Perianal Fistulas (INSPIRE)	Alofisel-5003 EUPAS24267	Phase IV	Recruiting	Gastrointestinal (Other)	Crohn's Disease and Complex Perianal Fistulas	Mesenchymal stem/stromal cells	No	N/A	Allogeneic	2018	St Mark's Hospital, London Northern General Hospital, Sheffield
Taysha Gene Therapies Inc	Safety and Efficacy of TSHA-102 in Pediatric Females with Rett Syndrome (Reveal Pediatric Study)	NCT06152237 TSHA-102-CL-102	Phase I/II	Recruiting	Neurological	Rett Syndrome	N/A	Yes, *in vivo*	AAV	N/A	2023	Guy's and St Thomas' NHS Foundation Trust, London
TC BioPharm Ltd	ACHIEVE - Efficacy and Effectiveness of Adoptive Cellular tHerapy wIth *ex vivo* Expanded Allogeneic ?d T-lymphocytes (TCB008) for Patients With Refractory or Relapsed Acute Myeloid Leukaemia (AML) (ACHIEVE)	TCB008-001 NCT05358808	Phase II/III	In planning/ set-up	Oncology (Haematology)	Refactory/ Relapsed Acute Myeloid Leukaemia	T cells	No	N/A	Allogeneic	2022	University Hospital of Wales, Cardiff Queen Elizabeth University Hospital, Glasgow Guy's and St Thomas' NHS Foundation Trust, London King's College Hospital NHS Foundation Trust, London The Royal Marsden Hospital NHS Foundation Trust, London
The Robert Jones & Agnes Hunt Orthopaedic Hospital NHS Foundation Trust	Autologous Stem Cells, Chondrocytes or the Two	R5242 A789 EudraCT-2010-022072-31 2010-022072-31 IRAS 50345 18480 19429 21156 MR/L010453/1 R5242A789	Phase II	In follow-up	Musculoskeletal	Osteoarthritis	Chondrocytes	No	N/A	Autologous	2013	The Robert Jones and Agnes Hunt Orthopaedic Hospital NHS Foundation Trust, Oswestry
The University of Manchester	A Phase I-II Study of Cryopreserved Autologous CD34+ Haematopoietic Stem Cells Transduced *ex vivo* with CD11b Lentiviral Vector Encoding Human Ids Tagged with Apoeii in Patients with Neuronopathic Mucopolysaccharidosis Type II (Nmps II, Hunters Syndrome)	ISRCTN12458940 2021-000400-38 R125432 IRAS 1004283 NCT05665166 EudraCT-2021-000400-38 GDCT0490401 GDC30033365 1004283	Phase I/II	Recruiting	Metabolic (Other)	Mucopolysaccharidosis II (MPS II) (Hunter Syndrome)	CD34 and/or CD133 stem cells	Yes, *ex vivo*	Lentivirus	Autologous	2023	Manchester University NHS Foundation Trust, Manchester
The University of Manchester Royal Manchester Children's Hospital	Gene Therapy with Modified Autologous Hematopoietic Stem Cells for Patients with Mucopolysaccharidosis Type IIIA	NCT04201405 R119861 UK-0239 2019-002051-42 EudraCT-2019-002051-42	Phase I/II	Completed	Metabolic (Other)	Mucopolysaccharidosis IIIA (MPS IIIA) (Sanfilippo Syndrome) - Lysosomal storage disease	CD34 and/or CD133 stem cells	Yes, *ex vivo*	Lentivirus	Autologous	2020	Manchester University NHS Foundation Trust, Manchester Royal Manchester Children's Hospital, Manchester Great Ormond Street Hospital for Children NHS Foundation Trust, London King's College Hospital NHS Foundation Trust, London University College London Hospitals NHS Foundation Trust, London University Hospitals Coventry and Warwickshire NHS Foundation Trust, Coventry
T-Knife GmbH	A Phase I/ II, First-in-Human, Open-Label, Accelerated-Titration, Two-Part Clinical Trial of TK-8001 in Patients With HLA-A*02:01 Genotype and Advanced-Stage/ Metastatic MAGE-A1+ Solid Tumors (IMAG1NE)	NCT05430555 TK-8001-01 2021-004158-49 Eudra-CT-2021-004158-49 NL83771.000.23 NL-OMON53302 EUCTR2021-004158-49-NL 2021-004158-49 (EudraCT Number)	Phase I/II	Terminated	Oncology (Other)	Solid Tumor	T cells	Yes, *ex vivo*	Retrovirus	Autologous	2022	The Christie NHS Foundation Trust, Manchester

Cell and Gene Therapy Catapult ATMP Clinical Trials Database 2024

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)
Transgene SA	A Clinical Trial Evaluating TG4050 in Head and Neck Cancer	NCT04183166	Phase I	Recruiting	Oncology (Other)	Squamous Cell Carcinoma of the Head and Neck	N/A	Yes, *in vivo*	MVA	N/A	2019	The Clatterbridge Cancer Centre NHS Foundation Trust, Liverpool Aintree University Hospital NHS Fondation Trust, Liverpool
Trizell Ltd	Efficacy & Safety of rAd-IFN Administered with Celecoxib & Gemcitabine in Patients with Malignant Pleural Mesothelioma (INFINITE)	rAd-IFN-MM-301 2017-003169-82 EudraCT-2017-003169-82 US-1791 1807-1791 17041 CRUK 17041 NL67688.000.19 EUCTR2017-003169-82-NL NL-OMON48042 2017-003169-82 (EudraCT Number)	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 University Hospital Plymouth NHS Trust, Plymouth The Royal Marsden NHS Foundation Trust, London Guy's and St Thomas' NHS Foundation Trust, London Wythenshawe Hospital, Manchester Churchill Hospital, Oxford
Ultragenyx Pharmaceutical Inc	Long Term Follow up to Evaluate DTX301 in Adults with Late-onset Otc Deficiency (CAPtivate)	301OTC02 IND-17190 NCT03636438 2018-000156-18 217067 2022-501146-30	Phase I/II	In follow-up	Metabolic (Other)	Ornithine -Transcarbamylase Deficiency	N/A	Yes, *in vivo*	AAV	N/A	2018	Queen Elizabeth Hospital, Birmingham
Ultragenyx Pharmaceutical Inc	Clinical Study of DTX301 AAV- Mediated Gene Transfer for Ornithine Transcarbamylase(OTC) Deficiency	2020-003384-25 EudraCT-2020-003384-25 DTX301-CL301 NCT05345171 jRCT2073220097 2024-514337-38-00 EUCT-2024-514337-38-00 2024-514337-38-00 (EUCT Number) IND 17190 2020-003384-25 (EudraCT Number) 2024-514337-38-00 (EU Trial (CTIS) Number)	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	Study of UX701 Gene Transfer for the Treatment of Wilson Disease	NCT04884815 UX701-CL301 2020-005266-34 21-000496 EudraCT-2020-005266-34 GDC30027882 GDCT0421865 2022-502873-40-00 EUCT-2022-502873-40-00 2022-502873-40-00 (EUCT Number) 2020-005266-34 (EudraCT Number)	Phase I/II	In follow-up	Genetic Disorders	Wilson Disease	N/A	Yes, *in vivo*	AAV	N/A	2021	King's College Hospital NHS Foundation Trust, London
UniQure NV	Safety and Efficacy of AMT-130 in European Adults With Early Manifest Huntington's Disease	CT-AMT-130-02 EudraCT-2020-001461-36 2020-001461-36 NCT05243017	Phase I/II	In follow-up	Neurological	Huntington Disease	N/A	Yes, *in vivo*	AAV	N/A	2021	University Hospital of Wales, Cardiff National Hospital for Neurology and Neurosurgery, London
University College London	CARPALL: Immunotherapy With CD19/22 CAR T-cells for CD19+ Haematological Malignancies	UCL 14/0529 2015-001144-10 EudraCT-2015-001144-10 IRAS 179225 GDC30021653 GDCT0366450 14118 CRUK-14118	Phase I	In follow-up	Oncology (Haematology)	Haematological Malignancies	T cells	Yes, *ex vivo*	Lentivirus	Autologous	2016	Royal Manchester Children's Hospital, Manchester Great Ormond Street Hospital for Children NHS Foundation Trust, London University College London Hospitals NHS Foundation Trust, London

Cell and Gene Therapy Catapult ATMP Clinical Trials Database 2024

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	Targeted Stem Cells Expressing Trail as a Therapy for Lung Cancer	UCL/14/0453	Phase I/II	Recruiting	Oncology (Other)	Non-Small Cell Lung Cancer	Mesenchymal stem/stromal cells	Yes, *ex vivo*	Lentivirus	Allogeneic	2019	University College London Hospitals NHS Foundation Trust, London
University College London	Gene Therapy for Haemophilia A (GO-8)	MR/L013185/1 2016-000925-38 EudraCT-2016-000925-38 UCL 13/0076 UCL13/0076 IRAS-207751 XX-0038	Phase I/II	Recruiting	Haematological	Hemophilia A (Factor VIII Deficiency)	N/A	Yes, *in vivo*	AAV	N/A	2017	Royal Free Hospital, London
University College London	Immunotherapy with Tacrolimus Resistant EBV CTL for Lymphoproliferative Disease after Solid Organ Transplant (ITREC)	UCL/16/0529 2017-001020-22 EudraCT 2017-001020-22 IRAS-225911	Phase I	In follow-up	Oncology (Haematology)	Post-Transplant Lymphoproliferative Disorder	T cells	Yes, *ex vivo*	Retrovirus	Autologous	2019	Great Ormond Street Hospital for Children NHS Foundation Trust, London King's College Hospital NHS Foundation Trust, London
University College London	A New Study Evaluating the Activity of Modular CAR T for Myeloma (MCARTY)	NCT04795882 UCL 129642	Phase I	In follow-up	Oncology (Haematology)	Multiple Myeloma	T cells	Yes, *ex vivo*	Lentivirus	Autologous	2022	University College London Hospitals NHS Foundation Trust, London
University College London	Immunotherapy Using CAR T-cells to Target CD19 for Relapsed/Refractory CD19+ Primary CNS Lymphoma (CAROUSEL)	UCL/126892	Phase I	In follow-up	Oncology (Other)	Primary CNS Lymphoma	T cells	Yes, *ex vivo*	Unknown	Autologous	2021	University College London Hospitals NHS Foundation Trust, London
University College London	Halting Ornithine Transcarbamylase Deficiency With Recombinant AAV in Children (HORACE)	18/0123 ISRCTN47106904 2020-005342-41 IRAS 1003541 EudraCT-2020-005342-41	Phase I/II	Recruiting	Genetic Disorders	Ornithine-Transcarbamylase Deficiency	N/A	Yes, *in vivo*	AAV	N/A	2023	Great Ormond Street Hospital for Children NHS Foundation Trust, London
University College London	Determining the Mechanisms of Loss of CAR T Cell Persistence (CARPERS)	UCL/156503	Phase IV	Recruiting	Oncology (Haematology)	Acute Lymphoblastic Leukaemia	Dendritic cells	Yes, *ex vivo*	N/A	Autologous	2023	Great Ormond Street Hospital for Children NHS Foundation Trust, London University College London Hospitals NHS Foundation Trust, London Royal Manchester Children's Hospital, Manchester

Cell and Gene Therapy Catapult ATMP Clinical Trials Database 2024

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	CAR T Cells to Target GD2 for DMG (CARMIGO)	UCL/ 150853	Phase I	Recruiting	Oncology (Other)	Glioma	T cells	Yes, *ex vivo*	Retrovirus	Autologous	2023	Great Ormond Street Hospital for Children NHS Foundation Trust, London
University College London	A Trial To Test CAR-T Cells For Glioblastoma In Adults When Standard Treatment Is Not Effective Or Stops Working	2022-003747-10 1007305 IRAS-1007305 UCL/126897 CPMS 57214 IRAS 1007305 EudraCT-2022-003747-10	Phase I	In planning/ set-up	Oncology (Other)	Glioblastoma Multiforme or Recurrent Glioblastoma Multiforme	T cells	Yes, *ex vivo*	Unknown	Unknown	2024	University College London Hospitals NHS Foundation Trust, London
University College London	A CAR T Study For Paediatric-Type Diffuse High-Grade Gliomas Including Diffuse Midline Glioma	1008429 IRAS-1008429 UCL/150463 IRAS 1008429	Phase I	Recruiting	Metabolic (Other)	High-Grade Glioma	T cells	Yes, *ex vivo*	Unknown	Autologous	2024	Great Ormond Street Hospital for Children NHS Foundation Trust, London
University College London University College London Hospitals NHS Foundation Trust	Immunotherapy for High Risk/Relapsed CD19+ Acute Lymphoblastic Leukaemia, B-cell Non-Hodgkin's Lymphoma (B-NHL) and Chronic Lymphocytic Leukaemia (CLL)/ Small Lymphocytic Lymphoma (SLL) Using CAR T-cells to Target CD19 (ALLCAR19)	UCL/16/0530 2016-004027-22 IRAS-217367 EudraCT-2016-004027-22	Phase I	In follow-up	Oncology (Haematology)	Acute Lymphocytic Leukemia	T cells	Yes, *ex vivo*	Lentivirus	Autologous	2017	University College London Hospitals NHS Foundation Trust, London
University of Birmingham	Selected Mesenchymal Stromal Cells to Reduce Inflammation in Patients with PSC and AIH (Merlin)	RG-13-124 WP8 GDC30011048 GDCT0266794 ISRCTN73586959 2016-000181-36 EudraCT-2016-000181-36 33534	Phase II	In follow-up	Gastrointestinal (Liver)	Primary Sclerosing Cholangitis, and Autoimmune Hepatitis	Mesenchymal stem/stromal cells	No	N/A	Allogeneic	2018	Queen Elizabeth Hospital, Birmingham
University of Birmingham	A Global Study of Novel Agents in Paediatric and Adolescent Relapsed and Refractory B-cell Non-Hodgkin Lymphoma (Glo-BNHL)	2024-510575-38 NCT05991388 RG_21-124 ITCC-100 1004701	Phase II/III	Recruiting	Oncology (Haematology)	B-Cell Non-Hodgkin Lymphoma	T cells	Yes, *ex vivo*	Lentivirus	Autologous	2024	Birmingham Children's Hospital, Birmingham Bristol Royal Hospital for Children, Bristol Royal Manchester Children's Hospital, Manchester
University of Birmingham	A Clinical Trial to See If a Mesenchymal Stem Cells Treatment Called Orbcel-C Can Help to Treat Primary Sclerosing Cholangitis, Rheumatoid Arthritis, Lupus Nephritis and Crohn's Disease	2019-003404-13 272535 IRAS 272535 CPMS 49716 EudraCT-2019-003404-13	Phase II	Recruiting	Inflammatory and immune system	Primary Sclerosing Cholangitis Rheumatoid Arthritis Lupus Nephritis Crohn's Disease (Regional Enteritis)	Mesenchymal stem/stromal cells	No	N/A	Allogeneic	2022	Queen Elizabeth Hospital, Birmingham Queens Medical Centre, Nottingham University Hospital, Nottingham Royal Victoria Infirmary, Newcastle Southampton General Hospital, Southampton

Cell and Gene Therapy Catapult ATMP Clinical Trials Database 2024

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 California Los Angeles	An Observational LTFU Study for Patients Previously Treated with Autologous *ex vivo* Gene Therapy for ADA-SCID	OTL-101-6	Phase III	Recruiting	Inflammatory and immune system	Adenosine Deaminase Deficiency Related Severe Combined Immunodeficiency Disease	CD34 and/or CD133 stem cells	Yes, *ex vivo*	Lentivirus	Autologous	2019	Great Ormond Street Hospital for Children NHS Foundation Trust, London
University of Oxford	TWO study: Cell Therapy Trial in Renal Transplantation	2017-001421-41 EudraCT-2017-001421-41 37306 12926 MR/N027930/1 TWOProtocolv1.0 227287 IRAS 227287	Phase II	Recruiting	Inflammatory and immune system	Kidney Transplant Rejection	T cells	No	N/A	Autologous	2016	Oxford University Hospitals NHS Foundation Trust, Oxford
Vertex Pharmaceuticals Inc	A Safety and Efficacy Study Evaluating CTX001 in Subjects with Transfusion-dependent ß-thalassemia	CTX001-111 2017-003351-38 EudraCT-2017-003351-38 NCT03655678 NCI-2020-01001 XX-0076 study-111 2024-516894-57-00 EUCT-2024-516894-57-00	Phase I/II	In follow-up	Haematological	Transfusion-Dependent β Thalassemia	CD34 and/or CD133 stem cells	Yes, *ex vivo*	Non-viral	Autologous	2018	Hammersmith Hospital, London University College London Hospitals NHS Foundation Trust, London
Vertex Pharmaceuticals Inc	A Safety and Efficacy Study Evaluating CTX001 in Subjects With Severe Sickle Cell Disease	CTX001-121 NCT03745287 2018-001320-19 EudraCT-2018-001320-19 XX-0081 study 121 2024-516067-83-00 EUCT-2024-516067-83-00	Phase II/III	Completed	Haematological	Severe Sickle Cell Disease	CD34 and/or CD133 stem cells	Yes, *ex vivo*	Non-viral	Autologous	2018	Imperial College Healthcare NHS Foundation Trust, London Barts Health NHS Trust, London
Vertex Pharmaceuticals Inc	A Long-term Follow-up Study in Subjects Who Received CTX001	CTX001-131 2018-002935-88 EudraCT-2018-002935-88 VX18-CTX001-131 study 131 2018-002935-88 (EudraCT Number) 2024-512654-19-00	Phase III	Recruiting	Haematological	Severe Sickle Cell Disease Transfusion Dependant β-Thalassemia	CD34 and/or CD133 stem cells	Yes, *ex vivo*	Non-viral	Autologous	2021	Imperial College Healthcare NHS Foundation Trust, London Hammersmith Hospital, London
Vertex Pharmaceuticals Inc	Evaluation of Safety and Efficacy of CTX001 in Pediatric Participants With Transfusion-Dependent ß-Thalassemia (TDT)	2021-002172-39 VX21-CTX001-141 NCT05356195 2021-002172-39 (EudraCT Number) P/549/2021	Phase III	Recruiting	Genetic Disorders	Transfusion-Dependent β-Thalassemia	CD34 and/or CD133 stem cells	Yes, *ex vivo*	Non-viral	Autologous	2022	Great Ormond Street Hospital for Children NHS Foundation Trust, London St Mary's Hospital, London
Vertex Pharmaceuticals Inc	Evaluation of Safety and Efficacy of CTX001 in Pediatric Participants With Severe Sickle Cell Disease (SCD)	2021-002173-26 VX21-CTX001-151 NCT05329649 study 151 2021-002173-26 (EudraCT Number) P/548/2021	Phase III	Recruiting	Haematological	Severe Sickle Cell Disease	CD34 and/or CD133 stem cells	Yes, *ex vivo*	Non-viral	Autologous	2022	St Mary's Hospital, London

Cell and Gene Therapy Catapult ATMP Clinical Trials Database 2024

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 Safety, Tolerability, and Efficacy Study of VX-264 in Participants with Type 1 Diabetes	2022-003318-35 EudraCT-2022-003318-35 VX22-264-101 NCT05791201 SNCTP000005450 BASEC2022-D0127 2024-515583-32-00 EUCT-2024-515583-32-00 2024-515583-32-00 (EU Trial (CTIS) Number) EUCTR2022-003318-35	Phase I/II	Recruiting	Metabolic (Diabetes)	Type 1 Diabetes	CD34 and/or CD133 stem cells	No	N/A	Allogeneic	2023	Churchill Hospital, Oxford Royal Victoria Infirmary, Newcastle Upon Tyne
Vertex Pharmaceuticals Inc	A Phase I/II Study of VX-522 in Participants with Cystic Fibrosis (CF)	NCT05668741 VX21-522-001 2022-000726-25 2023-504786-23-00 2022-000726-25 (EudraCT Number) 2023-504786-23-00 (Other Identifier: EU CT Number) EUCT-2023-504786-23-00	Phase I/II	Recruiting	Respiratory	Cystic Fibrosis	N/A	Yes, in vivo	Non-viral	N/A	2023	Royal Papworth Hospital NHS Foundation Trust, Cambridge Royal Hospital for Children, Glasgow Royal Brompton Hospital, London Wythenshawe Hospital, Manchester All Wales Adult Cystic Fibrosis Centre, University Hospital Llandough, Penarth Southampton General Hospital, Southampton
Verve Therapeutics Inc	A Study of VERVE-102 in Patients With Familial Hypercholesterolemia or Premature Coronary Artery Disease	NCT06164730 VT-10201	Phase I	Recruiting	Metabolic (Other)	Heterozygous Familial Hypercholesterolemia Coronary Artery Disease	N/A	Yes, in vivo	Non-viral	N/A	2024	Clinical Study Site, London Clinical Study Site, Manchester
Verve Therapeutics Inc	A Study of VERVE-101 in Patients with Familial Hypercholesterolemia and Cardiovascular Disease	NCT05398029 VT-1001 GDC30031492 GDCT0467180	Phase I	In planning/ set-up	Metabolic (Other)	Heterozygous Familial Hypercholesterolemia, Atherosclerotic Cardiovascular Disease, and Uncontrolled Hypercholesterolemia	N/A	Yes, in vivo	Non-viral	N/A	2024	Unconfirmed UK sites
Vivet Therapeutics SAS	A Phase I/II Study of VTX-801 in Adult Patients with Wilson's Disease (GATEWAY)	NCT04537377 VTX-801_CLN_001 EudraCT-2020-000963-22	Phase I/II	In follow-up	Metabolic (Other)	Wilson Disease	N/A	Yes, in vivo	AAV	N/A	2021	Royal Surrey NHS Foundation Trust, Guildford