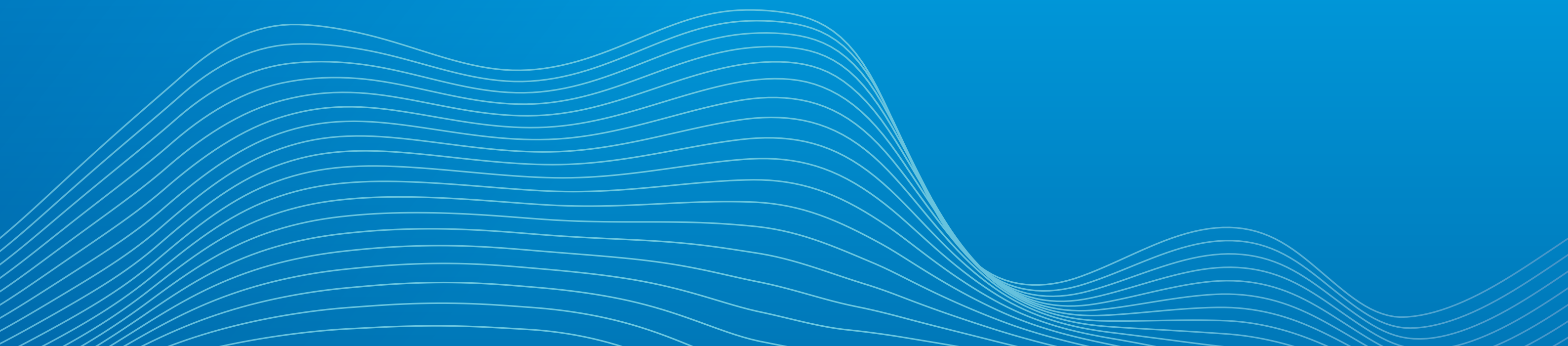


Cell and Gene Therapy Catapult

UK 2025 ATMP Clinical Trials Database

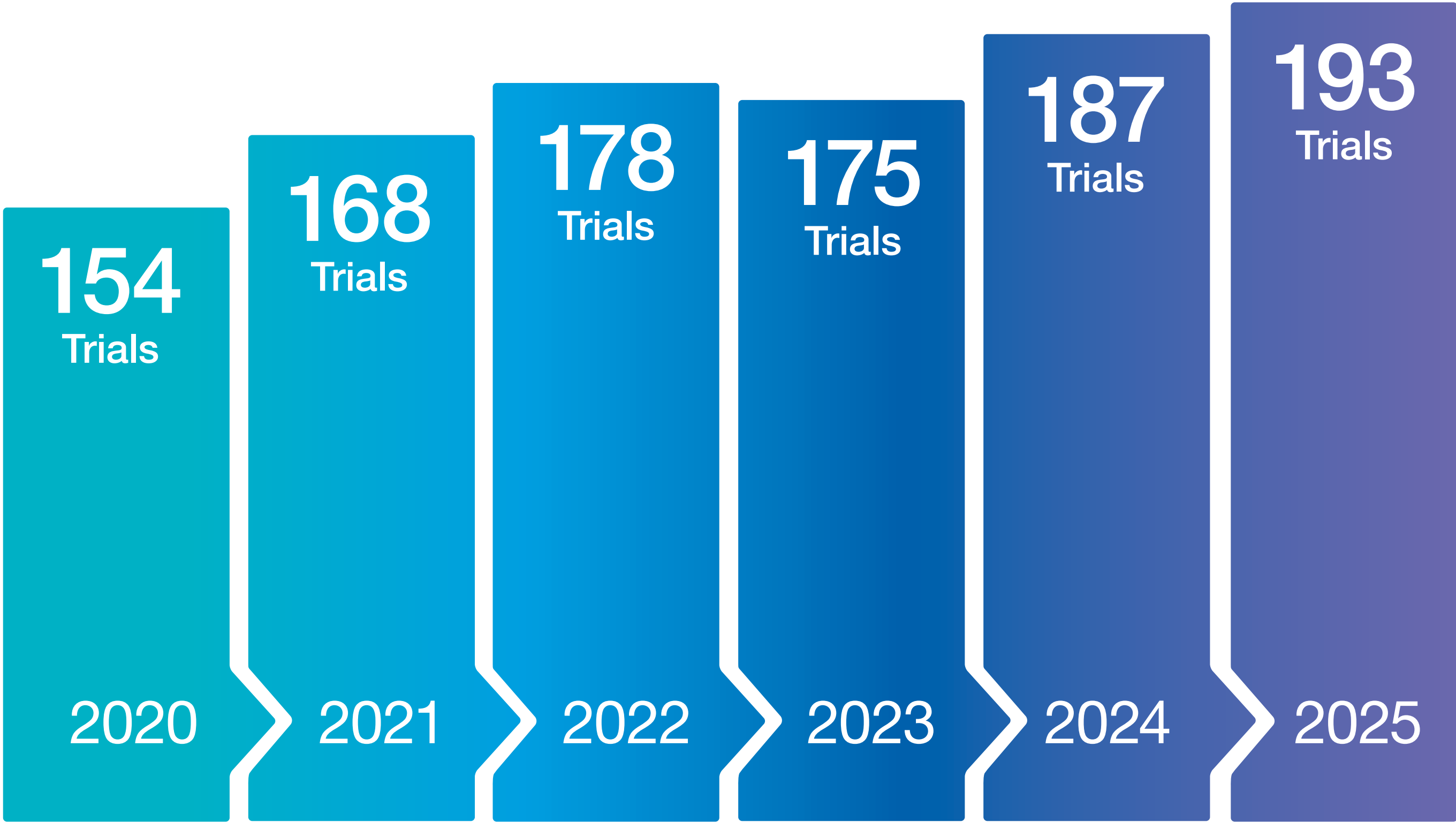
This clinical trial database covers all ATMP trials taking place in the UK in 2025.

Here, we highlight key data from the database ►



Strong and sustained UK clinical trial capability

Ongoing clinical trials in the UK have increased (193 in 2025 vs 187 in 2024).

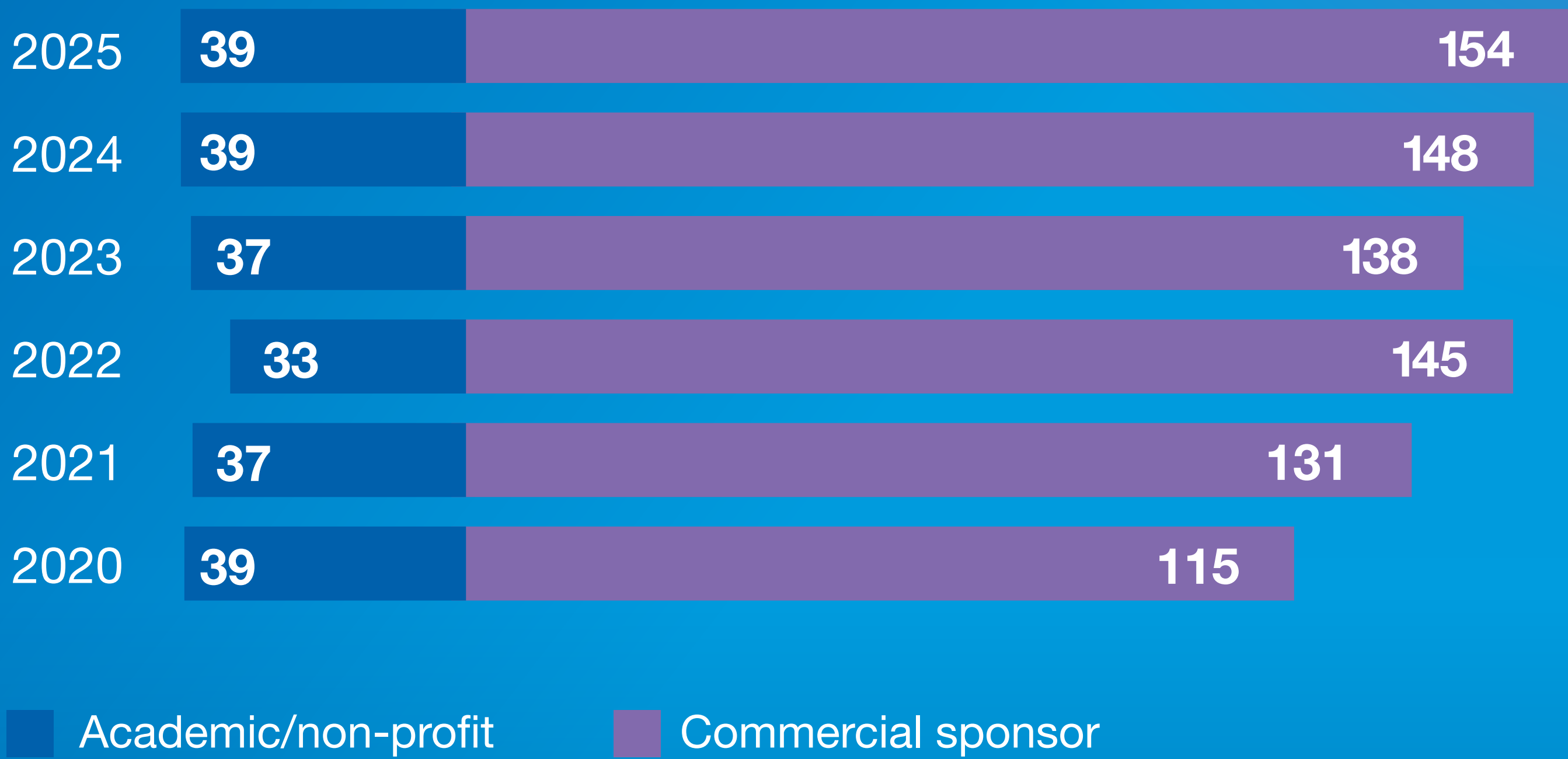


*Alliance for Regenerative Medicine (ARM) 2025 Cell and Gene Therapy Clinical Trials report

UK remains an attractive location for commercially sponsored ATMP trials



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



The UK participates in over half of all European trials

57%

of European trials
include UK
participation*

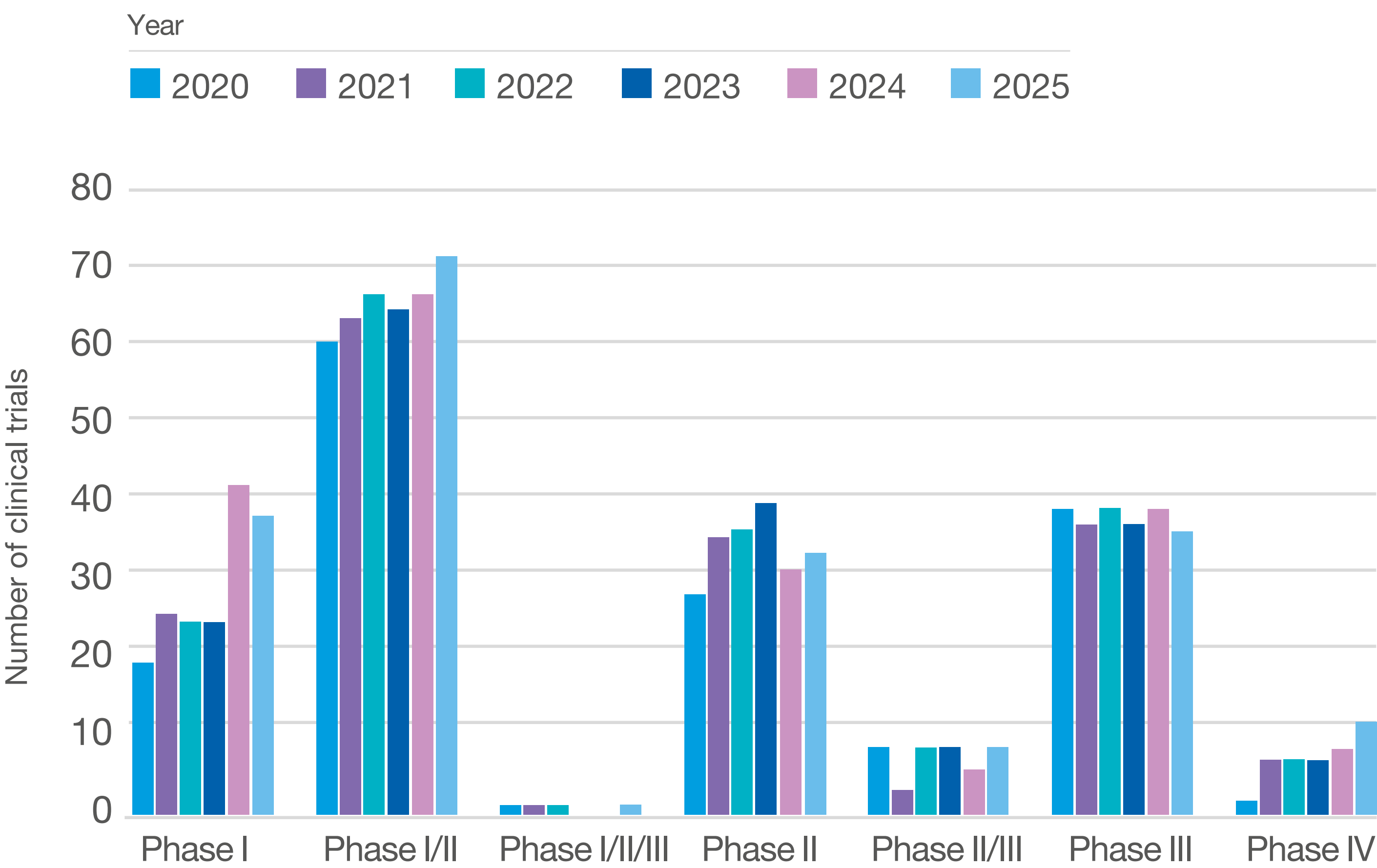
193
Trials

337
Trials

*Alliance for Regenerative Medicine (ARM) 2025 Cell and Gene Therapy Clinical Trials report

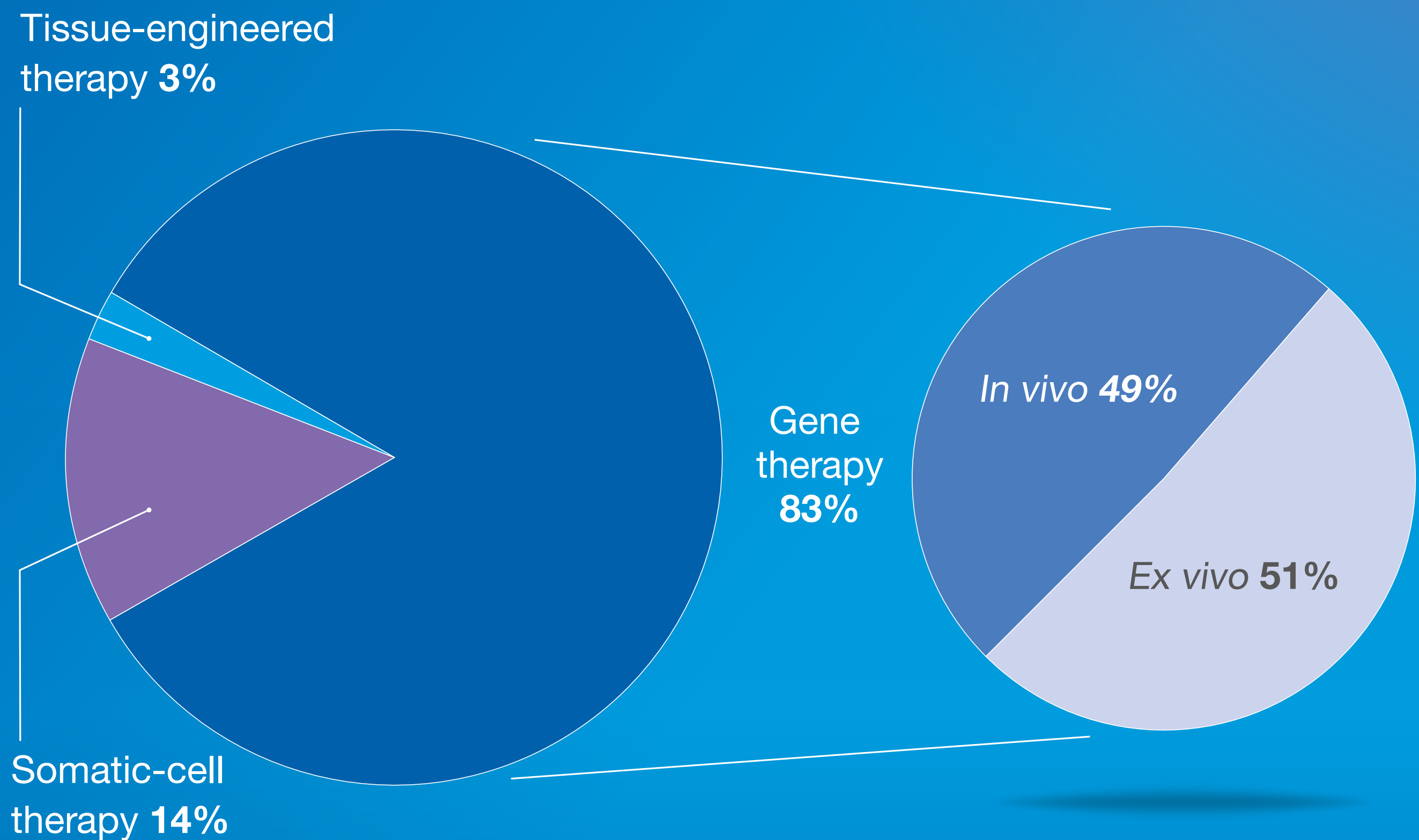
Growth of early-phase innovation in ATMP clinical trials

High early-phase trial activity has been maintained, with strong progression into later phases and an uplift in phase I/II trials.



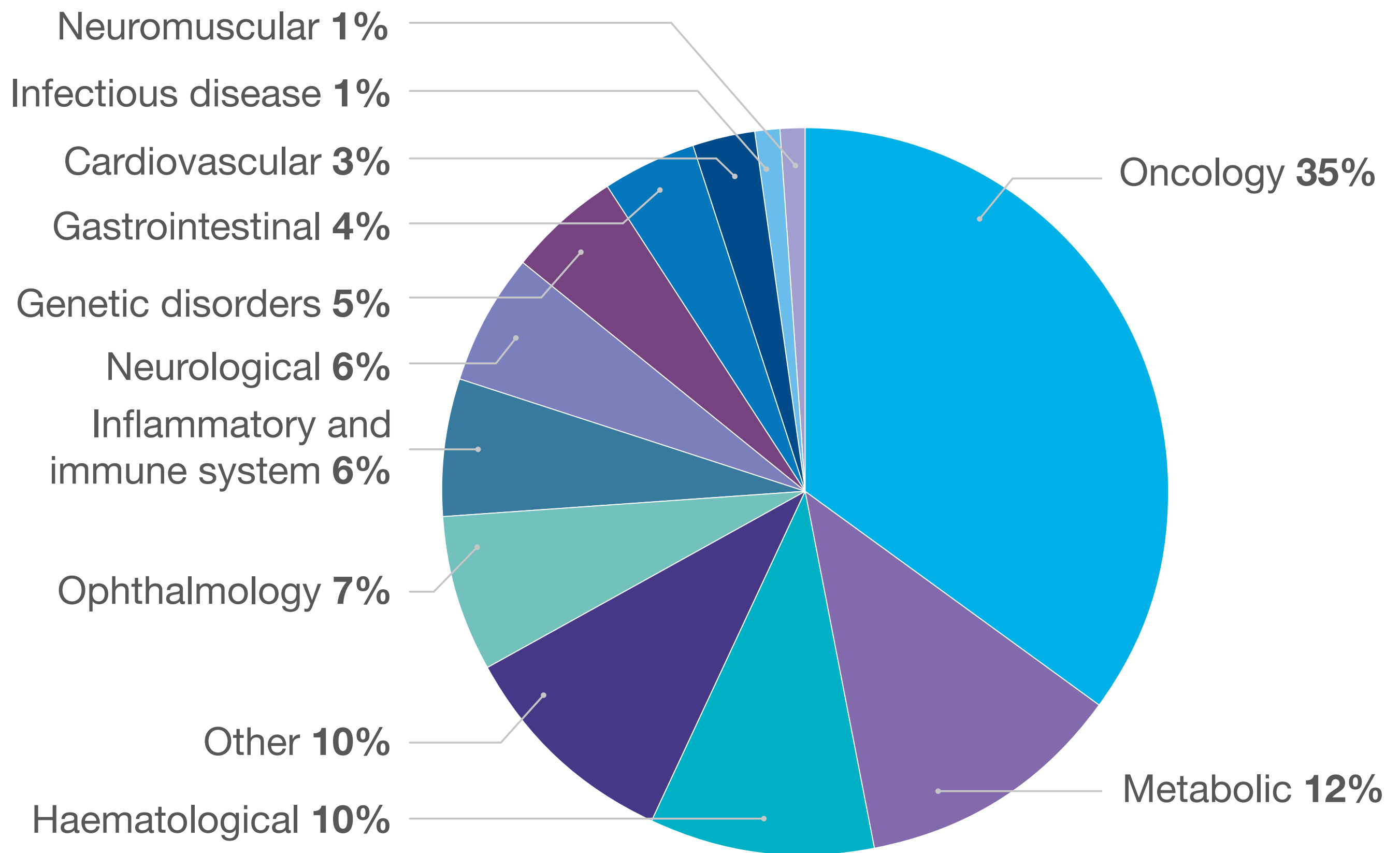
The majority of trials continue to involve genetically modified therapies

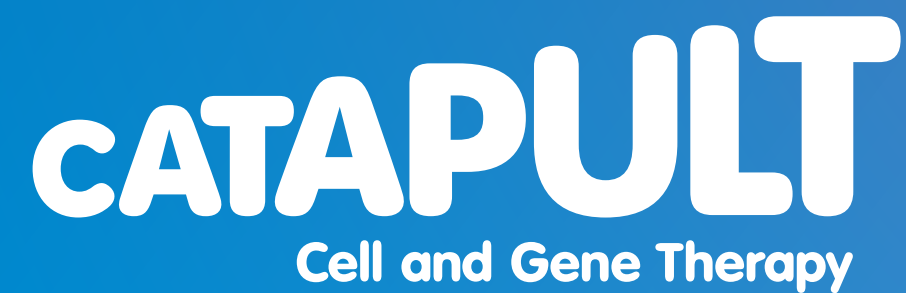
With an even distribution between *in vivo* and *ex vivo* gene therapy trials.



Oncology remains the dominant therapeutic area under investigation

There is promising growth in other disease areas such as inflammation and the immune system.





Read on for the full 2025 Cell and Gene Therapy Catapult ATMP Clinical Trials Database

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	Study of Subretinally Injected AAVB-081 in Patients With Usher Syndrome Type IB (USH1B) Retinitis Pigmentosa	EudraCT-2022-001092-14 TIGEM5_USH 1009616 EUCT-2024-518489-29-00 GDC30035840 081-101 GDCT0523487	Phase I/II	Recruiting	Ophthalmology	Usher Syndrome Type Ib (Ush1b) Retinitis Pigmentosa	N/A	Yes, <i>in vivo</i>	AAV	N/A	2024	Moorfields Eye Hospital NHS Foundation Trust, London Retina Clinic, London
AbbVie Inc	Pivotal 2 Study of RGX-314 Gene Therapy in Participants With nAMD (ASCENT)	jRCT2073240017 M23-409 NCT05407636 RGX-314-3101 EUCT-2023-503666-23-00 IRAS 1008138 RGX-314-3101/M23-409	Phase III	In follow-up	Ophthalmology	Neovascular (Wet) Age-related Macular Degeneration	N/A	Yes, <i>in vivo</i>	AAV	N/A	2022	Liverpool University Hospitals NHS Foundation Trust, Liverpool Moorfields Eye Hospital NHS Foundation Trust, London Retina Clinic, London Oxford University Hospitals NHS Foundation Trust, Oxford University Hospital Southampton NHS Foundation Trust, Southampton Stoke Mandeville Hospital, Aylesbury Hammersmith Hospital, London Bristol Eye Hospital, Bristol Manchester University NHS Foundation Trust, Manchester Royal Victoria Infirmary, Newcastle Upon Tyne Sunderland Eye Infirmary, Sunderland Gloucestershire Royal Hospital, Gloucester Leeds Teaching Hospitals NHS Trust, Leeds Bradford Royal Infirmary, Bradford
Accession Therapeutics Ltd	A Study of ATTR-01 in Participants with Select Epithelial Solid Tumours (ATTEST)	1010660 ATTR-01-01 IRAS 1010660 ISRCTN38972074 CPMS 57739 EUCT-2024-516722-59-00 NCT06977737	Phase I/II	Recruiting	Oncology (Other)	Breast Cancer, Lung Cancer, Ovarian Cancer, Bile Duct Cancer (Cholangiocarcinoma), Non-Small Cell Lung Cancer, Epithelial Tumor, Head and Neck Cancer, Pancreatic Cancer, Bladder Cancer, Solid Tumor, Endometrial Cancer, Carcinomas, Transitional Cell Cancer (Urothelial Cell Cancer)	N/A	No	Oncolytic Virus	N/A	2025	Churchill Hospital, Oxford University Hospitals NHS Foundation Trust, Oxford Beatson West of Scotland Cancer Centre, Glasgow Velindre Cancer Centre, Cardiff St James' University Hospital, Leeds
Achilles Therapeutics Plc	ATL001 in Patients with Metastatic or Recurrent Melanoma	NCT03997474 ATX-ME-001 EudraCT-2018-003446-16 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 Upon Tyne Cambridge University Hospitals NHS Foundation Trust, Cambridge University College London Hospitals NHS Foundation Trust, London Guys and St Thomas' NHS Foundation Trust, London The Royal Marsden Hospital NHS Foundation Trust, London University Hospital Southampton NHS Foundation Trust, Southampton
Achilles Therapeutics Plc	ATL001 in Patients with Advanced Unresectable or Metastatic NSCLC	NCT04032847 ATX-NS-001 EudraCT-2018-001005-85	Phase I/II	Terminated	Oncology (Other)	Non-Small Cell Lung Cancer	T cells	No	N/A	Autologous	2019	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 The Leeds Teaching Hospitals NHS Trust, Leeds Guys and St Thomas' NHS Foundation Trust, London
Achilles Therapeutics Plc	Long-Term Follow-Up Study of Patients Receiving ATL001	ATX-LTFU-001	Phase II	Terminated	Oncology (Other)	Metastatic or Recurrent Melanoma Non-Small Cell Lung Cancer	T cells	No	N/A	Autologous	2022	University College London Hospitals NHS Foundation Trust, London

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	MAGE A10c796T for Advanced NSCLC	NCT02592577 ADP 0022-003 NCI-2016-00533 CDR78041715303 2015-0805 IRAS-216896 2016-002518-28	Phase I	Completed	Oncology (Other)	Non-Small Cell Lung Cancer	T cells	Yes, ex vivo	Lentivirus	Autologous	2015	University College London Hospitals NHS Foundation Trust, London The Christie NHS Foundation Trust, Manchester
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 NCI-2019-05288 XX-0091 19292 OSU-19245 201907126 NL-OMON52674 NL70428.000.19 2024-513032-14	Phase II	In follow-up	Oncology (Other)	Synovial Sarcoma	T cells	Yes, ex vivo	Lentivirus	Autologous	2019	The Royal Marsden Hospital NHS Foundation Trust, London University College London Hospitals, NHS Foundation Trust, London The Christie NHS Foundation Trust, Manchester
Adaptimmune Therapeutics Plc	AFPC332T in Advanced HCC	16111GCC 20160692 201903120 ADP-0033-001 CDR789757 EUCT-2024-514437-37-00 EudraCT-2017-000778-12 GDC30009397 GDCT0254617 NCI-2017-01188 UMGCC16111GCC XX-0056	Phase I	Completed	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 UCLH Clinical Research Facility, London The Christie NHS Foundation Trust, Manchester
Adaptimmune Therapeutics Plc	Spearhead 1 Study in Subjects with Advanced Synovial Sarcoma or Myxoid/Round Cell Liposarcoma	NCT04044768 ADP 0044-002 NCI-2019-05482 EudraCT-2019-000589-39 20251 19194 OSU-19130 201904212 RG1005467 19-004764 CRUK-17621 EUCT-2024-512847-21-00	Phase II	In follow-up	Oncology (Other)	Soft Tissue Sarcoma, Sarcomas, Myxoid Liposarcoma, Round Cell Liposarcoma, Synovial Sarcoma	T cells	Yes, ex vivo	Lentivirus	Autologous	2019	UCLH Cancer Clinical Trials Unit, London The Christie NHS Foundation Trust, Manchester
Adaptimmune Therapeutics Plc	ADP-A2M4CD8 in HLA-A2+ Subjects With MAGE-A4 Positive Esophageal or Esophagogastric Junction Cancers (SURPASS-2)	NCT04752358 ADP-0055-002 EudraCT-2020-005802-24	Phase II	Terminated	Oncology (Other)	Oesophageal Cancer	T cells	Yes, ex vivo	Lentivirus	Autologous	2021	Christie NHS Foundation Trust, Manchester Beatson West of Scotland Cancer Centre, Glasgow University College London Hospitals NHS Foundation Trust, London Guy's and St Thomas' NHS Foundation Trust, London
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 ADP-0055-003/GOG-3084 NCI-2023-02480	Phase II	In follow-up	Oncology (Other)	Head And Neck Cancer Squamous Cell Carcinoma Oral Cavity (Mouth) Cancer Oropharyngeal Cancer Hypopharyngeal Cancer Laryngeal Cancer Recurrent Head And Neck Cancer Squamous Cell Carcinoma	T cells	Yes, ex vivo	Lentivirus	Autologous	2023	University College London Hospitals NHS Foundation Trust, London The Royal Marsden Hospital NHS Foundation Trust, London The Christie NHS Foundation Trust, Manchester
Adaptimmune Therapeutics 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 Hospital NHS Foundation Trust, London University College Hospitals NHS Foundation Trust, London Christie Hospital NHS Foundation Trust, Manchester

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	Study to Evaluate Safety and Antitumor Activity of Lete-cel (GSK3377794) in HLA-A2+ Participants with NY-ESO-1 Positive Previously Treated Advanced (Metastatic or Unresectable) Synovial Sarcoma or Myxoid/Round Cell Liposarcoma	208467 Substudy 2	Phase II	In follow-up	Oncology (Other)	Myxoid Liposarcoma, Round Cell Liposarcoma, Synovial Sarcoma	T cells	Yes, ex vivo	Lentivirus	Autologous	2019	The Royal Marsden Hospital NHS Foundation Trust, London The Christie NHS Foundation Trust, Manchester University College London Hospitals NHS Foundation Trust, London
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 CPMS 53941 EudraCT-2022-002261-15 2538429-16-0	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 2020-004735-25 IRAS-290504	Phase I	Completed	Oncology (Other)	Squamous Cell Carcinoma of the Head and Neck	N/A	Yes, in vivo	Oncolytic Virus	N/A	2021	Cardiff & Vale University Health Board, Cardiff The Clatterbridge Cancer Centre NHS Foundation Trust, Liverpool The Royal Marsden Hospital 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 IRAS-1004337 2021-005570-26 EUCT-2023-503525-19-00	Phase I	Completed	Oncology (Other)	Squamous Cell Carcinoma, Epithelial Tumor, Metastatic Cancer, Recurrent Head And Neck 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)	1005052 CPMS 52187 EudraCT-2022-000335-22 IRAS 1005052 ISRCTN94964671 MK-3475-C84 NG-350A-02 NCI-2022-02429 EUCT-2023-503457-35-00 2021-1163	Phase I	In follow-up	Oncology (Other)	Multiple Myeloma (Kahler Disease)	N/A	Yes, in vivo	Adenovirus	N/A	2022	Churchill Hospital, Oxford University Hospitals NHS Foundation Trust, Oxford The Clatterbridge Cancer Centre NHS Foundation Trust, Liverpool
Akamis Bio Ltd	NG-350A Plus Chemoradiotherapy for Locally Advanced Rectal Cancer (FORTRESS)	NG-350A-03 ISRCTN12595783 IRAS-1011106	Phase I	Recruiting	Oncology (Other)	Rectal cancer	N/A	Yes, in vivo	Oncolytic Virus	N/A	2025	University College London Hospitals NHS Foundation Trust, London The Royal Marsden Hospital NHS Foundation Trust, Sutton
Akouos Inc	Gene Therapy Trial for Otoferlin Gene-mediated Hearing Loss	NCT05821959 AK-OTOF-101 22-019935 56570 160180 EUCT-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 of London Hospitals NHS Foundation Trust, London

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)
Alexion Pharmaceuticals Inc	A Phase IB/II Study of CAR T Cell Therapy Targeting CD19 and BCMA in Participants With Relapsed or Refractory AL Amyloidosis (ALACRITY)	D831AC00001	Phase I/II	Recruiting	Oncology (Haematology)	Replaced or Refractory AL Amyloidosis	T cells	Yes, <i>ex vivo</i>	Lentivirus	Autologous	2025	Royal Free London NHS Foundation Trust, London
Amgen Inc	Pembrolizumab With Talimogene Laherparepvec or Placebo in Unresected Melanoma (MASTERKEY-265)	20110265 EudraCT-2014-000185-22 NCT02263508 CDR765511 NCI-2015-00094 GDC30007789 GDCT0243919 BBS-B-GT34 16276 CT682 P 39916 20160320 XX-0030 13832	Phase III	Terminated	Oncology (Other)	Melanoma	N/A	Yes, <i>in vivo</i>	Oncolytic Virus	N/A	2014	Queen Elizabeth Hospital, Birmingham Royal Surrey Country Hospital, Guildford St James's University Hospital, Leeds Leicester Royal Infirmary, Leicester Guy's Hospital, Guy's and St Thomas' NHS Foundation Trust, London The Royal Marsden Hospital NHS Foundation Trust, London The Christie NHS Foundation Trust, Manchester Churchill Hospital, Oxford University Hospitals NHS Foundation Trust, Oxford Royal Preston Hospital, Preston Southampton General Hospital, Southampton
Amgen Inc	Registry Study for Talimogene Laherparepvec	20120139 CDR762564 EudraCT-2015-003196-29 EUPAS43115	Phase IV	Completed	Oncology (Other)	Various Cancers	N/A	Yes, <i>in vivo</i>	Oncolytic Virus	N/A	2010	Guy's Hospital, Guy's and St Thomas' NHS Foundation Trust, London The Royal Marsden Hospital NHS Foundation Trust, London The Clatterbridge Cancer Centre NHS Foundation Trust, Liverpool
Amgen Inc	Single-arm Trial to Evaluate the Role of the Immune Response to Talimogene Laherparepvec in Unresected Melanoma (TVEC-325)	20120325 NCT02366195 TVEC-325 NCRN - 2960 UKCRN-17314 CTBE2016000319 NCI-2016-00727 CDR769527 IRAS ID 158013	Phase II	Completed	Oncology (Other)	Melanoma	N/A	Yes, <i>in vivo</i>	Oncolytic Virus	N/A	2015	Guy's Hospital, Guy's and St Thomas' NHS Foundation Trust, London The Clatterbridge Cancer Centre NHS Foundation Trust, Liverpool
Amgen Inc	Postmarketing Prospective Study of Melanoma Patients Treated with Imlygic to Characterize Risk of Herpetic Infection	20130193 EUPAS15128 NCI-2019-06388 2018-00720	Phase IV	In follow-up	Oncology (Other)	Melanoma	N/A	Yes, <i>in vivo</i>	Oncolytic Virus	N/A	2017	Cheltenham General Hospital, Cheltenham Guy's Hospital, Guy's and St Thomas' NHS Foundation Trust, London The Royal Marsden Hospital NHS Foundation Trust, London Churchill Hospital, Oxford University Hospitals NHS Foundation Trust, Oxford Musgrove Park Hospital, Taunton
Amgen Inc	Talimogene Laherparepvec With Pembrolizumab in Melanoma Following Progression on Prior Anti-PD-1 Based Therapy (MASTERKEY-115) (Mk-3475-A07/KEYNOTE-A07)	20180115 NCI-2019-07131 EudraCT-2019-001906-61 NL70568.000.19	Phase II	Completed	Oncology (Other)	Melanoma	N/A	Yes, <i>in vivo</i>	Oncolytic Virus	N/A	2020	Guys and St Thomas' NHS Foundation Trust, London The Royal Marsden Hospital NHS Foundation Trust, London
Amphera BV	Study on Dendritic Cell Therapy as Maintenance Therapy for Patients That Have Been Treated for Mesothelioma	MM04 NCT03610360 GDC20010660 GDCT0277700 2017-001774-41	Phase II/III	Completed	Oncology (Other)	Malignant Pleural Mesothelioma Malignant Mesothelioma	Antigen presenting cells	No	N/A	Autologous	2018	University of Leicester, Leicester

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)
AskBio Inc	Phosphatase Inhibition by Intracoronary Gene Therapy in Subjects With Non-Ischemic NYHA Class III Heart Failure (GenePHIT)	NCT05598333 ASK-CHF2-CS201 EUCT-2024-510581-17-00 1009799 IRAS 1009799	Phase II	In follow-up	Cardiovascular	Congestive Heart Failure (Heart Failure)	N/A	No	AAV	N/A	2023	University Hospital of Wales, Cardiff NHS Golden Jubilee, Glasgow National Heart and Lung Institute, Imperial College London, London Manchester Royal Infirmary, Manchester Leeds General Infirmary, Leeds
AskBio Inc	A Study of AAV2-GDNF in Adults with Moderate Parkinson's Disease (REGENERATE-PD) (REGENERATE-PD)	NCT06285643 ASK-PD5-CS201 EUCT-2023-506519-16-00	Phase II	Recruiting	Neurological	Parkinson's Disease	N/A	Yes, <i>in vivo</i>	AAV	N/A	2024	Southmead Hospital, North Bristol NHS Trust, Bristol University Hospital of Wales, Cardiff
Astellas Gene Therapies	Gene Transfer Clinical Study in Crigler-Najjar Syndrome (VALENS)	NCT03223194 AT342-02 EudraCT-2017-001631-39 MOH_2018-04-29_002345 US-1589	Phase I/II	Terminated	Metabolic (Other)	Crigler-Najjar Syndrome	N/A	Yes, <i>in vivo</i>	AAV	N/A	2017	King's College Hospital NHS Foundation Trust, London
Astellas Gene Therapies	Gene Transfer Study in Patients with Late Onset Pompe Disease (FORTIS)	AT845-01 EudraCT-2019-003595-38	Phase I/II	In follow-up	Metabolic (Other)	Pompe Disease	N/A	Yes, <i>in vivo</i>	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 EudraCT-2016-005245-23 239878	Phase I/II	Recruiting	Ophthalmology	Age-related Macular Degeneration (AMD)	Retinal	No	N/A	Allogeneic	2018	Site GB44001, London
AstraZeneca Plc	A Study of MEDI5395 in Combination with Durvalumab in Participants with Select Advanced Solid Tumors	D6450C00001 NCI-2019-07042 82519 EudraCT-2017-000851-16	Phase I	Completed	Oncology (Other)	Breast Cancer, Renal Cell Carcinoma, Non-Small Cell Lung Cancer, Colorectal Cancer, Melanoma, Solid Tumor, Head And Neck Squamous Cell Carcinoma (HNSC), Hepatocellular Carcinoma	N/A	Yes, <i>in vivo</i>	Oncolytic Virus	N/A	2019	St James's University Hospital, Leeds The Royal Marsden Hospital NHS Foundation Trust, London
Atamyo Therapeutics SAS	ATA-100 (Formerly GNT0006) Gene Therapy Trial in Patients with LGMDR9	ATA-001-FKRP NCT05224505 GDC30029488 GDCT0441697 EudraCT-2021-004276-33 EUCT-2023-506677-36-00 306837	Phase I	In follow-up	Musculoskeletal	FKRP-related Limb-girdle Muscular Dystrophy (LGMDR9, Formerly LGMD2I)	N/A	Yes, <i>in vivo</i>	AAV	N/A	2022	Royal Victoria Infirmary, Newcastle Upon Tyne

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)
Atolus Therapeutics Plc	Phase I/II Study Evaluating AUTO4 in Patients with TRBC1 Positive T Cell Lymphoma	NCT03590574 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, The Newcastle Upon Tyne Hospitals NHS Foundation Trust, Newcastle Upon Tyne
Atolus Therapeutics Plc	Cluster of Differentiation Antigen 19/22(CD19/22) CAR T Cells (AUTO3) for the Treatment of Diffuse Large B Cell Lymphoma	NCT03287817 2016-004682-11 UKCTG-10549 NCI-2019-05093 19307 201907085 20190669	Phase I/II	Terminated	Oncology (Haematology)	Lymphoma, Diffuse Large B-Cell Lymphoma, Primary Mediastinal B-Cell Lymphoma	T cells	Yes, ex vivo	Retrovirus	Autologous	2017	Freeman Hospital NHS Foundation Trust, Newcastle Upon Tyne Manchester University NHS Foundation Trust, Manchester University College London Hospitals NHS Foundation Trust, London The Beatson West of Scotland Cancer Centre, Glasgow
Atolus Therapeutics Plc	CD19 /22 CAR T Cells (AUTO3) for the Treatment of B Cell Acute Lymphoblastic Leukemia (ALL) (AMELIA)	AUTO3-PA1 NCT03289455 2016-004680-39 IRAS-220370	Phase I/II	Completed	Oncology (Haematology)	B-Cell Acute Lymphocytic Leukemia (B-Cell Acute Lymphoblastic Leukaemia)	T cells	Yes, ex vivo	Retrovirus	Autologous	2017	Great Ormond Street Hospital for Children NHS Foundation Trust, London University College London Hospitals NHS Foundation Trust, London Royal Manchester Children's Hospital, Manchester
Atolus Therapeutics Plc	Long-term Follow-up of Patients Treated with Genetically Modified Autologous T Cells	AUTO-LT1 EudraCT-2016-004867-38 20231175 EUCT-2024-511684-28-00	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 Upon Tyne
Atolus Therapeutics Plc	A Study of CD19 Targeted CAR T Cell Therapy in Adult Patients with Relapsed or Refractory B Cell Acute Lymphoblastic Leukaemia (ALL)	NCT04404660 NCI-2020-04370 202004253 FELIX (AUTO1-AL1) 17562 CRUK-17562 GDC30020798 GDCT0356178 EudraCT-2019-001937-16 EUCT-2024-512903-38-00	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, The Newcastle Upon Tyne Hospitals NHS Foundation Trust, Newcastle Upon Tyne University Hospitals Birmingham NHS Foundation Trust, Birmingham
Atolus Therapeutics Plc	A Study of CD19 Targeted Car T Cell Therapy in Patients with Severe, Refractory Systemic Lupus Erythematosus (SLE) (CARLYSE)	NCT06333483 AUTO1-SL1 EUCT-2023-508236-60-00	Phase I	Recruiting	Inflammatory and immune system	Systemic Lupus Erythematosus	T cells	Yes, ex vivo	Lentivirus	Autologous	2024	Great Ormond Street Hospital for Children, NHS Foundation Trust, London University College London Hospitals NHS Foundation Trust, London Manchester Royal Infirmary, Manchester University NHS Foundation Trust, Manchester
Atolus Therapeutics Plc	A Study of CD19 Targeted CAR T Cell Therapy in Pediatric Patients with Relapsed or Refractory B Cell Acute Lymphoblastic Leukemia (B ALL) and Aggressive Mature B-cell Non-hodgkin Lymphoma (B NHL)	AUTO1-PY1 2023-506307-26-00 PY01 1007600	Phase I/II	Recruiting	Oncology (Haematology)	B-Cell Acute Lymphocytic Leukemia (B-Cell Acute Lymphoblastic Leukaemia), Burkitt Lymphoma, B-Cell Non-Hodgkin Lymphoma, Acute Lymphocytic Leukemia (ALL, Acute Lymphoblastic Leukemia), Diffuse Large B-Cell Lymphoma, Primary Mediastinal B-Cell 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 Royal Manchester Children's Hospital, Manchester

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	Obe-cel in Refractory Progressive Forms of Multiple Sclerosis (BOBCAT)	EUCT-2024-519552-93-00 AUTO1-MS1 NCT07139743 1011413	Phase I	Recruiting	Neurological	Multiple Sclerosis	T cells	Yes, ex vivo	Lentivirus	Autologous	2025	University College London Hospitals NHS Foundation Trust, London
AviadoBio Ltd	A Study to Evaluate the Safety and Effect of AVB-101, a Gene Therapy Product, in Subjects with a Genetic Sub-type of Frontotemporal Dementia (FTD-GRN)	2022-002568-62 AVB-PGRN-001 NCT06064890 NL82614.000.22 U1111-1301-8705 EUCT-2023-509444-10-00 NL-OMON53802 IRAS-1005928	Phase I/II	Recruiting	Neurological	Dementia, Frontotemporal Dementia (Pick Disease)	N/A	Yes, in vivo	AAV	N/A	2023	Cambridge University Hospitals NHS Foundation Trust, Cambridge University College London Hospital NHS Foundation Trust, London University Hospital of Wales, Cardiff
Axovia Therapeutics Ltd	Gene Therapy Study to Assess the Safety, Tolerability and Effectiveness of AXV-101 When Injected into the Eye in Patients with a Mutated BBS1 Gene to Prevent Sight Loss	GDC30035064 ISRCTN96250868 1011952 CLIN101-AXV-101(SR) CPMS 67544 IRAS 1011952	Phase I	Recruiting	Genetic Disorders	Retinitis Pigmentosa, Nyctalopia (Night Blindness) due to Vitamin A Deficiency, Retinal Degeneration, Unspecified Genetic Disorders	N/A	Yes, in vivo	AAV	N/A	2025	Great Ormond Street Hospital for Children NHS Foundation Trust, London St Thomas' Hospital, Guys and St Thomas' NHS Foundation Trust, London
Azellon Ltd	A Clinical Trial to Test the Safety of Cell Bandage, a Product Made from the Patient's Own Bone Marrow Stem Cells, in the Treatment of Meniscus (Cartilage) Tears in the Knee	ACTLP001-01 UKCRN-12776 IRAS-98378 12/SC/0198	Phase I/II	Completed	Musculoskeletal	Knee Meniscus Repair	Mesenchymal stem/stromal cells	No	N/A	Autologous	2012	Southmead Hospital, Bristol
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	BAY19429 NCT03588299 EudraCT-2017-000806-39 1708-1646 US-1646 BAY2599023/19429 NL-OMON55861 NL66590.000.18 EUCT-2023-505827-29-00	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	In follow-up	Ophthalmology	X-Linked Retinitis Pigmentosa	N/A	Yes, in vivo	AAV	N/A	2024	Moorfields Eye Hospital NHS Foundation Trust, London Retina Clinic, London Oxford Eye Hospital, Oxford University Hospitals (OUH) NHS Foundation Trust, 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 EUCT-2023-509256-34-00 1009231	Phase I/II	Recruiting	Genetic Disorders	Alpha-1 Antitrypsin Deficiency	N/A	Yes, in vivo	Non-viral	N/A	2024	Clinical Study Centre, London

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)
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 227090 40531	Phase I/II	In follow-up	Respiratory	Coronavirus Disease 2019 (COVID-19), Coronavirus Disease 2019 (COVID-19) Associated Acute Respiratory Distress Syndrome, Acute Respiratory Distress Syndrome	Mesenchymal Stem/Stromal Cells	No	N/A	Allogeneic	2019	Royal Victoria Hospital, Belfast
Bellicum Pharmaceuticals Inc	Safety Study of Gene Modified Donor T-cells Following TCRαβ+ Depleted Stem Cell Transplant	EudraCT-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 London NHS Foundation Trust, London The Newcastle Upon Tyne Hospitals NHS Foundation Trust, Newcastle Upon Tyne
Biogen Inc	Long-term Safety and Efficacy Follow-up of BLIB111 for the Treatment of Choroideremia and BLIB112 for the Treatment of X-Linked Retinitis Pigmentosa (SOLSTICE)	273CH201 NSR-CHM-OS2 EudraCT-2017-003104-42 EUCT-2023-507994-16-00 NL69445.000.21 GDCT0310530	Phase III	Recruiting	Ophthalmology	Choroideremia, X-Linked Retinitis Pigmentosa	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
Biogen Inc	Efficacy and Safety of BLIB111 for the Treatment of Choroideremia (STAR)	2015-003958-41 NCT03496012 IRAS-193561 20151100 273CH301	Phase III	Completed	Ophthalmology	Choroideremia	N/A	Yes, in vivo	AAV	N/A	2017	Manchester Royal Infirmary, Manchester John Radcliffe Hospital, Oxford
Biogen Inc	A Clinical Trial of Retinal Gene Therapy for X-linked Retinitis Pigmentosa Using BLIB112 (XIRIUS)	NCT03116113 2016-003852-60 IRAS-214620 GDCT0338239 GDC30019031 274RP101 (NSR-RPGR-01)	Phase II/III	Completed	Ophthalmology	X-Linked Retinitis Pigmentosa	N/A	Yes, in vivo	AAV	N/A	2017	Manchester Royal Eye Hospital, Manchester Oxford Eye Hospital, Oxford Research Site, Southampton
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 EudraCT-2022-001246-38 IRAS-1005791 270-401 (GENEr8-LTE) U1111-1297-8403 EUCT-2023-507749-27-00	Phase IV	Recruiting	Haematological	Heamophilia A	N/A	Yes, in vivo	AAV	N/A	2023	Addenbrooke's Hospital, Cambridge Hammersmith Hospital, London Queen Elizabeth Hospital, Birmingham Royal London Hospital, London St Thomas' Hospital, Guys and St Thomas' NHS Foundation Trust, London University Hospital Southampton NHS Foundation Trust, Southampton
BioMarin Pharmaceutical Inc	Gene Therapy Study in Severe Haemophilia A Patients (270-201)	270-201 GDC40001726 GDCT0211369 BMN270-201 NCT02576795 UKCRN-19239 HAEM 4448 IRAS 173490 UKCTG-5877 BMN 270-201 EudraCT-2014-003880-38	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 Basingstoke and North Hampshire Hospital, Basingstoke Imperial College Healthcare NHS Trust, London University Hospitals Bristol and Weston NHS Foundation Trust, Bristol NHS Greater Glasgow and Clyde, Glasgow

Sponsor	Title	Clinical Database Numbers	Trial Phase	Trial Status	Disease Area	Indication	Cell Type	Gene Modification / Gene Therapy	Type of Vector Used (if applicable)	Autologous/ Allogeneic	Year Trial Started	United Kingdom Site(s)
BioMarin Pharmaceutical Inc	Single-Arm Study To Evaluate The Efficacy and Safety of Valoctocogene Roxaparvec in Hemophilia A Patients (BMN 270-301) (BMN 270-301)	Study 270-301 NCT03370913 EudraCT-2017-003215-19 XX-0071	Phase III	Completed	Haematological	Hemophilia A (Factor VIII Deficiency)	N/A	Yes, <i>in vivo</i>	AAV	N/A	2017	Queen Elizabeth Hospital, Birmingham Addenbrooke's Hospital, Cambridge Glasgow Royal Infirmary, Glasgow Barts and The London School of Medicine and Dentistry, London Hammersmith Hospital, London St Thomas' Hospital, Guys and St Thomas' NHS Foundation Trust, London Churchill Hospital, Oxford University Hospitals NHS Foundation Trust, Oxford University Hospital Southampton, Southampton Royal Cornwall Hospital, Truro
BioMarin Pharmaceutical Inc	Single-arm Study To Evaluate The Efficacy and Safety of Valoctocogene Roxaparvec in Hemophilia A Patients at A Dose of 4E13 vg/kg (GENEr8-2)	Study 270-302 NCT03392974 EudraCT-2017-003573-34 XX-0080	Phase III	Completed	Haematological	Hemophilia A (Factor VIII Deficiency)	N/A	Yes, <i>in vivo</i>	AAV	N/A	2018	Addenbrooke's Hospital, Cambridge Glasgow Royal Infirmary, Glasgow Barts and The London School of Medicine and Dentistry, London Hammersmith Hospital, London St Thomas' Hospital, Guys and St Thomas' NHS Foundation Trust, London Churchill Hospital, Oxford University Hospitals NHS Foundation Trust, Oxford University Hospital Southampton NHS Foundation Trust, Southampton Queen Elizabeth Hospital NHS Foundation Trust, Birmingham
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 UK-0233 202200029 GDCT0447396 Study 270-203	Phase I/II	Terminated	Haematological	Hemophilia A (Factor VIII Deficiency)	N/A	Yes, <i>in vivo</i>	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 EudraCT-2019-001878-28 BMN 307-201 NCT04480567	Phase I/II	In follow-up	Metabolic (Other)	Phenylketonuria (PKU)	N/A	Yes, <i>in vivo</i>	AAV	N/A	2020	University Hospital Birmingham NHS Foundation Trust, Birmingham
BioNTech SE	Safety and Preliminary Efficacy Trial of BNT142 in Patients with CLDN6-positive Solid Tumors	4781/0029 BNT142-01 EUCT-2024-512639-58-00 EudraCT-2021-005481-18 NCT05262530 NL-OMON53910 NL83219.000.23	Phase I/II	In follow-up	Oncology (Other)	Advanced Solid Tumors	N/A	Yes, <i>in vivo</i>	N/A	N/A	2022	Cambridge University Hospitals NHS Foundation Trust, Cambridge The Clatterbridge Cancer Centre NHS Foundation Trust, Liverpool Guy's & St Thomas' NHS Foundation Trust, London Hammersmith Hospital, London Sarah Cannon Research Institute, London Nottingham University Hospitals NHS Trust, Nottingham Churchill Hospital, Oxford University Hospitals NHS Foundation Trust, Oxford
Boehringer Ingelheim International GmbH	A Long-term Study to Monitor the Health Status of People with Cystic Fibrosis who Took Part in a Previous Study with BI 3720931 (Lenticlair™-ON)	EUCT-2023-504909-37-00 1504-0003 U1111-1291-2932 NCT06962852	Phase I/II	Recruiting	Respiratory	Cystic Fibrosis	N/A	Yes, <i>in vivo</i>	Lentivirus	N/A	2025	Queen Elizabeth Hospital Birmingham, Birmingham Royal Brompton & Harefield Hospitals, London Wythenshawe Hospital, Manchester University Hospital Llandough, Penarth
Boston Children's Hospital	Gene Transfer for Severe Combined Immunodeficiency, X-linked (SCID-X1) Using a Self-inactivating (SIN) Gammaretroviral Vector	IND14067	Phase I/II	Completed	Inflammatory and immune system	X-Linked SCID Severe Combined Immune Deficiency (SCID)	CD34 and/or CD133 stem cells	Yes, <i>ex vivo</i>	Retrovirus	Autologous	2010	Great Ormond Street Hospital for Children NHS Foundation Trust, London University College London Hospitals NHS Foundation Trust, London

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)
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 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
Bristol-Myers Squibb Co	A Study to Compare the Efficacy and Safety of BMS-986393 Versus Standard Regimens in Adult Participants With Relapsed or Refractory and Lenalidomide-exposed Multiple Myeloma (QUINTESENTIAL-2)	CA088-1007 NCI-2025-00729 U1111-1308-9347 EUCT-2024-515279-37-00 SNCTP000006359 BASEC2024-02357 JRCT2053250007 CA0881007 202500756	Phase III	Recruiting	Oncology (Haematology)	Lanalidomide-refractory Multiple Myeloma	T cells	Yes, ex vivo	Lentivirus	Autologous	2025	The Christie NHS Foundation Trust, Manchester Newcastle Hospitals NHS Foundation Trust, Newcastle Upon Tyne Addenbrooke's Hospital, Cambridge University College London Hospitals NHS Foundation Trust, London Churchill Hospital, Oxford University Hospitals NHS Foundation Trust, Oxford
Cabaletta Bio Inc	RESET-Myositis: An Open-Label Study to Evaluate the Safety and Efficacy of CABA-201 in Subjects With Active Idiopathic Inflammatory Myopathy or Juvenile Idiopathic Inflammatory Myopathy	NCT06154252 CAB-201-002 IRAS 1008852 ISRCTN75158615 CPMS 58719	Phase I/II	Recruiting	Musculoskeletal	Active Idiopathic Inflammatory Myopathy or Juvenile Idiopathic Inflammatory Myopathy	T cells	Yes, ex vivo	Lentivirus	Autologous	2023	King's College Hospital NHS Foundation Trust, London Salford Royal Hospital, Salford Manchester Royal Infirmary, Manchester University College London Hospitals NHS Foundation Trust, London
Cancer Research UK	A Trial of ChAdOx1 and MVA Vaccines Against MAGE-A3 and NY-ESO-1	CRUKD/20/001 17221 CUKD/20/001 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 University Hospitals NHS Foundation Trust, 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 Research UK	AST-VAC2 Vaccine in Patients with Non-small Cell Lung Cancer	NCT03371485 CRUKD/17/003 IRAS-218704 GDCT0307919 15137 EudraCT-2016-002577-35	Phase I	Completed	Oncology (Other)	Non-Small Cell Lung Cancer	Antigen presenting cells	No	N/A	Allogeneic	2018	Southampton General Hospital, Southampton Queen Elizabeth Hospital Birmingham, Birmingham
Cancer Research UK	A Phase I/II Trial of Anti-GD2 T-cells (1RG-CART)	CRUKD15001 152527 CRUKD/15/001 9177 EudraCT-2013-004554-17	Phase I	Completed	Oncology (Other)	Relapsed or Refractory Neuroblastoma	T cells	Yes, ex vivo	Retrovirus	Autologous	2016	Great Ormond Street Hospital For Children NHS Foundation Trust, London University College London (UCL), London
Cancer Research UK	A Phase I/II Trial of ALETA-001 for the Treatment of Participants with B-cell Malignancies	NCT06045910 CRUKD/23/001 IRAS-1007028	Phase I/II	Recruiting	Oncology (Haematology)	B-Cell Non-Hodgkin Lymphoma	T cells	Yes, ex vivo	Lentivirus	Autologous	2024	University Hospital Birmingham NHS Foundation Trust, Birmingham Cambridge University Hospitals, Cambridge St James's University Hospital, Leeds University College London Hospitals NHS Foundation Trust, London Manhester Royal Infirmary, Manchester The Christie NHS Foundation Trust, Manchester The Royal Marsden Hospital NHS Foundation Trust, Sutton

Sponsor	Title	Clinical Database Numbers	Trial Phase	Trial Status	Disease Area	Indication	Cell Type	Gene Modification / Gene Therapy	Type of Vector Used (if applicable)	Autologous/ Allogeneic	Year Trial Started	United Kingdom Site(s)
Cancer Research UK UCL Cancer Trials Centre	CAR-T Cells for Children, Teenagers and Young Adults with Sarcoma	1009001 UCL/150859 CPMS 60914	Phase I	Recruiting	Oncology (Other)	Rhabdomyosarcoma Ewing Sarcoma Desmoplastic Small Round Cell Tumour (DSRCT)	T cells	Yes, ex vivo	Unknown	Autologous	2024	University College London Hospitals NHS Foundation Trust, London Great Ormond Street Hospital for Children NHS Foundation Trust, London
Cancer Vaccines Ltd	Investigation of a Therapeutic Vaccine (ACIT-1) in Cancer	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	Royal Liverpool University Hospital, Liverpool The Clatterbridge Cancer Centre NHS Foundation Trust, Liverpool
Celgene Corp	A Study to Compare the Efficacy and Safety of JCAR017 to Standard of Care in Adult Subjects with High-risk, Transplant-eligible Relapsed or Refractory Aggressive B-cell Non-Hodgkin Lymphomas (TRANSFORM)	NCT03575351 JCAR017-BCM-003 U1111-1213-1944 EudraCT-2018-000929-32 2018-000929-32 NCI-2018-02503 JapicCTI-194718 XX-0058 18-455 SNCTP000003546 BASEC2019-00499 jRCT1080224646 2019-00499 NL65765.000.18 NL-OMON-52699 0451-0311	Phase III	Completed	Oncology (Haematology)	B-Cell Non-Hodgkin Lymphoma	T cells	Yes, ex vivo	Lentivirus	Autologous	2018	UCL Cancer Institute, London University Hospital Southampton NHS Foundation Trust, Southampton
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 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 bb2121-MM-003 280357 2020-00777 MM 003 EUCT-2023-509848-10-00	Phase III	In follow-up	Oncology (Haematology)	Multiple Myeloma	T cells	Yes, ex vivo	Lentivirus	Autologous	2019	The Leeds Teaching Hospitals NHS Trust, Leeds
Celgene Corp	Long-Term Follow-Up Protocol for Participants Treated With Gene-Modified T Cells	18082 CTIS2023-504201-36-00 EMA/409800/20212021 EudraCT-2017-001465-24 GC-LTFU-001 jRCT203230513 NCI-2018-00957 study GC-LTFU-001 U1111-1206-8250 2023-504201-36 (Other Identifier) (OTHER: EU CTR)	Phase II/III	Recruiting	Oncology (Other)	Neoplasms	T cells	Yes, ex vivo	Lentivirus	Autologous	2018	UCL Cancer Institute, London King's College Hospital NHS Foundation Trust, London The Christie NHS Foundation Trust, Manchester Somers Cancer Research, Southampton

Sponsor	Title	Clinical Database Numbers	Trial Phase	Trial Status	Disease Area	Indication	Cell Type	Gene Modification / Gene Therapy	Type of Vector Used (if applicable)	Autologous/ Allogeneic	Year Trial Started	United Kingdom Site(s)
Celgene Corp	Trial to Determine the Efficacy and Safety of JCAR017 in Adult Participants with Aggressive B-cell Non-hodgkin Lymphoma (TRANSCENDWORLD)	NCT03484702 JCAR017-BCM-001 U1111-1209-4055 2017-000106-38 EudraCT-2017-000106-38 JapicCTI-184129 JMA-IIA00387 XX-0049 BCM-001 JRCT2090220387 GC-LTFU-001 EUCTR2017-000106-38 BASEC2018-00628 SNCTP000003121 2017-000106-38-BE 2018-00628	Phase II	Completed	Oncology (Haematology)	B-Cell Non-Hodgkin Lymphoma	T cells	Yes, ex vivo	Lentivirus	Autologous	2018	UCL Cancer Institute, London The Christie NHS Foundation Trust, Manchester
Celgene Corp	An Efficacy and Safety Study of bb2121 in Subjects with Relapsed and Refractory Multiple Myeloma and in Subjects with High-risk Multiple Myeloma (KarMMA-2)	NCT03601078 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 2023-505183-10-00 EUCT-2023-505183-10-00 2023-505183-10 (Other Identifier) (OTHER: EU CT Number) 016664 IND 016664	Phase II	In follow-up	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)	NCT04245839 2024-510966-18 19-3011 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 EUCT-2024-510966-18-00 276831 JPRN-JRCT1080225154	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 Vicleucei 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)	202400148 CA089-1043 EUCT-2022-501346-30-00 EudraCT-2022-501346-30 GDCT0498724 JRCT2061230084 NCI-2024-01211 NCT06045806 NCT06399393 U1111-1280-9736 022482 IND 022482	Phase III	In follow-up	Oncology (Other)	Multiple Myeloma (Kahler Disease)	T cells	Yes, ex vivo	Lentivirus	Autologous	2023	Queen Elizabeth Hospital, Birmingham Local Institution - site 0081, Glasgow Local Institution - site 0143, Southampton King's College Hospital NHS Foundation Trust, London Hammersmith Hospital, London

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 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 Multicentre Observational Study to Assess Long-Term Outcome of Participants in the EXCELLENT Clinical Trial (PERFECT)	CPT-MI-R01 IRAS-343725 2024-A00423-44	Phase I/II	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
Chiesi Farmaceutici SpA	Efficacy and Safety of Autologous Cultivated Limbal Stem Cells Transplantation (ACL SCT) for Restoration of Corneal Epithelium in Patients with Limbal Stem Cell Deficiency (HOLOCORE)	CCD-GPLSCD01-03 2014-002845-23 IRAS-186764 SHOA1008 HLSTM03 P/199/2012	Phase IV	Completed	Ophthalmology	Limbal Stem Cell Deficiency	Corneal	No	N/A	Autologous	2015	Newcastle Upon Tyne Hospitals NHS Foundation Trust, Newcastle Upon Tyne Moorfields Eye Hospital NHS Foundation Trust, London University of Nottingham Queen's Medical Centre, Nottingham
Cook Group Inc	Autologous Cell Therapy for Treatment of Fecal Incontinence	NCT01600755 09-025 EudraCT-2013-004672-35 IRAS 141522	Phase I/II	Completed	Gastrointestinal (Other)	Fecal Incontinence	Other	No	N/A	Autologous	2013	Royal London Hospital, London
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	Phase III	In follow-up	Renal and Urogenital	Stress Urinary Incontinence	Skeletal Muscle	No	N/A	Autologous	2019	Derriford Hospital, Plymouth St James's University Hospital, Leeds
CSL Ltd	HOPE-B: Trial of AMT-061 in Severe or Moderately Severe Hemophilia B Patients	NCT03569891 EudraCT-2017-004305-40 00113507 B/BE/18/BVW7 1803-1742 US-1742 XX-0089 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	Completed	Haematological	Haemophilia B	N/A	Yes, <i>in vivo</i>	AAV	N/A	2018	University Hospitals Bristol NHS Foundation Trust, Bristol Addenbrooke's Hospital, Cambridge Royal London Hospital, London University Hospital Southampton NHS Foundation Trust, Southampton
CSL Ltd	Long-term Follow-up Study of Male Adults With Hemophilia B Previously Treated With Etranacogene Dezaparvovec (CSL222) (IX-TEND 3003)	CSL222_3003 EUCT-2023-503765-37-00 IX-TEND 3003	Phase II/III	Recruiting	Haematological	Haemophilia B (Factor IX Deficiency)	N/A	Yes, <i>ex vivo</i>	AAV	N/A	2023	Addenbrooke's Hospital, Cambridge University Hospital Southampton NHS Foundation Trust, Southampton Barts Health NHS Trust, London

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)
Cynata Therapeutics Ltd	A Study of CYP-001 for the Treatment of Steroid-resistant Acute Graft Versus Host Disease	CYP-GvHD-P1-01 EUCT-2016-000070-38 GDC30006526 UKCTG-37640 GDC30009532	Phase I	Completed	Inflammatory and immune system	Graft Versus Host Disease (GVHD)	Mesenchymal stem/stromal cells	No	N/A	Allogeneic	2017	Bristol and Weston NHS Foundation Trust, Bristol Leeds Teaching Hospitals NHS Trust, Leeds Liverpool University Hospitals NHS Foundation Trust, Liverpool The Christie Foundation Trust, Manchester Nottingham University NHS Foundation Trust, Nottingham
Eli Lilly and Co	Safety and Efficacy of Encapsulated Allogeneic FVIII Cell Therapy in Haemophilia A	EudraCT-2019-004210-33 SIG-001-121 NCT04541628	Phase I/II	Terminated	Haematological	Hemophilia A (Factor VIII Deficiency)	Retinal	Yes, ex vivo	Non-viral	Allogeneic	2020	Clinical Study Site, London Clinical Study Site, Southampton Manchester Royal Infirmary, Manchester
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 1008195	Phase I/II	Recruiting	Neurological	Dravet Syndrome (Severe Myoclonic Epilepsy of Infancy)	N/A	Yes, in vivo	AAV	N/A	2024	Queen Elizabeth Hospital, Glasgow Great Ormond Street Hospital for Children NHS Foundation Trust, London Sheffield Childrens Hospital, Sheffield
enGene Holdings Inc	LEGEND Study: EG-70 in NMIBC Patients BCG-Unresponsive and High-Risk NMIBC Incompletely Treated With BCG or BCG-Naïve	NCT04752722 EG-70-101 EUCT-2024-512900-20-00 202500129	Phase I/II	Recruiting	Oncology (Other)	Non Muscle Invasive Bladder Cancer (NMIBC) (Superficial Bladder Cancer)	N/A	Yes, in vivo	Non-viral	N/A	2021	The Royal Marsden Hospital NHS Foundation Trust, London
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 NCT07055321	Phase I	Completed	Respiratory	Asthma, Viral Respiratory Tract Infection	N/A	Yes, in vivo	Non-viral	N/A	2023	Simbec Research Limited, Pentrebach Merthyr Tydfil
Evelina London Children's Hospital Guy's and St Thomas' NHS Foundation Trust	An Observational Cohort Study to Evaluate the Efficacy and Safety of Onasemnogene Apeparovvec in Children with Spinal Muscular Atrophy Type 1: Real-world Evidence from 6 Infusion Centres in the United Kingdom	GDC20040356	Phase IV	Completed	Neurological	Spinal Muscular Atrophy (SMA)	N/A	Yes, in vivo	AAV	N/A	2021	Unconfirmed UK Sites
F. Hoffmann-La Roche Ltd	A Gene Delivery Study To Evaluate The Safety And Expression Of Delandistrogene Moxeparovvec In Participants Under The Age Of Four With Duchenne Muscular Dystrophy (DMD)	B/BE/22/BW5 BN43881 EMA-002677-PIP01-19 EUCT-2023-509901-57-00 EudraCT-2022-000691-19 NCT06128564 SRP-9001-302 Study 302 331338	Phase II	Suspended	Musculoskeletal	Duchenne Muscular Dystrophy	N/A	Yes, in vivo	AAV	N/A	2023	Great Ormond Street Hospital for Children NHS Foundation, London John Radcliffe Hospital, Oxford

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)
Fate Therapeutics Inc	A Phase I Study of FT819 in B-cell Mediated Autoimmune Disease	NCT06308978 FT819-102 EUCT-2024-517397-25-00	Phase I	Recruiting	Inflammatory and immune system	Systemic Lupus Erythematosus Lupus Nephritis	T cells	Yes, <i>ex vivo</i>	Non-viral	Allogeneic	2024	Manchester University Hospital NHS Foundation Trust, Manchester Cambridge University Hospital NHS Foundation Trust, Cambridge University College London Hospitals NHS Foundation Trust, London
Ferring Ventures Ltd	Efficacy & Safety of rAd-IFN Administered with Celecoxib & Gemcitabine in Patients with Malignant Pleural Mesothelioma (INFINITE)	rAd-IFN-MM-301 EudraCT-2017-003169-82 US-1791 1807-1791 17041 CRUK 17041 NL67688.000.19 NL-OMON48042	Phase III	Completed	Oncology (Other)	Malignant Pleural Mesothelioma	N/A	Yes, <i>in vivo</i>	Adenovirus	N/A	2019	Beatson West of Scotland Cancer Centre, Glasgow Derriford Hospital, Plymouth Guy's and St Thomas' NHS Foundation Trust, London Wythenshawe Hospital, Manchester Churchill Hospital, Oxford University Hospitals NHS Foundation Trust, Oxford The Royal Marsden Hospital NHS Foundation Trust, Sutton The Queen Elizabeth Hospital, London
Gamida Cell Ltd	Stem Cell Transplantation With NiCord (Omidubicel) vs Standard UCB in Patients With Leukemia, Lymphoma, and MDS	NCT02730299 GC P05.01.020 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 GCP#05.01.020 NL-OMON49347 EUCTR2016-000704-28-NL NL59195.000.16	Phase III	Completed	Oncology (Haematology)	Leukemia, Lymphoma, and Myelodysplastic Syndrome (MDS)	CD34 and/or CD133 stem cells	No	N/A	Allogeneic	2016	Queen Elizabeth Hospital, Birmingham Central Manchester University Hospitals NHS Foundation Trust, Manchester The Royal Marsden Hospital NHS Foundation Trust, London St James's University Hospital, Leeds
GEMMA Biotherapeutics Inc	Study of Safety, Tolerability and Efficacy of PBGM01 in Pediatric Participants with GM1 Gangliosidosis (Imagine-1)	EudraCT-2020-001109-22 PBGM01-001 NCT04713475 IRAS 284305	Phase I/II	In follow-up	Metabolic (Other)	GM1 Gangliosidosis	N/A	Yes, <i>in vivo</i>	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 UKCRN-13647 MREC-N-GTAC196 EudraCT-2012-000242-35 SNCTP000001301	Phase I/II	In follow-up	Genetic Disorders	Chronic Granulomatous Disease	CD34 and/or CD133 stem cells	Yes, <i>ex vivo</i>	Lentivirus	Autologous	2013	Great Ormond Street Hospital for Children NHS Foundation Trust, London University College London Hospitals NHS Foundation Trust, London Royal Free London 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 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, <i>ex vivo</i>	Lentivirus	Autologous	2014	UCL Institute of Child Health, London

Sponsor	Title	Clinical Database Numbers	Trial Phase	Trial Status	Disease Area	Indication	Cell Type	Gene Modification / Gene Therapy	Type of Vector Used (if applicable)	Autologous/ Allogeneic	Year Trial Started	United Kingdom Site(s)
Genethon SA	Microdystrophin (Gnt0004) Gene Therapy Clinical Trial in Duchenne Muscular Dystrophy a Phase I/II/III Study with a Dose Determination Part Followed by an Efficacy and Safety Evaluation, Quadruple Blind Placebo-controlled Part and Then by a Long Term Safety Follow up Part, in Ambulant Boys	EudraCT-2020-002093-27 GNT-016-MDYF 2023-505187-11-00 EUCT-2023-505187-11-00	Phase I	Recruiting	Musculoskeletal	Duchenne Muscular Dystrophy	N/A	Yes, in vivo	AAV	N/A	2021	Great Ormond Street Hospital for Children NHS Foundation Trust, London
Genetix Biotherapeutics Inc	A Study of the Efficacy and Safety of Hematopoietic Stem Cells Transduced with Lenti-D Lentiviral Vector for the Treatment of Cerebral Adrenoleukodystrophy (CALD)	ALD-102 EudraCT-2011-001953-10 MCRN3045 UKCRN -16192 091388 XX-0067 P/290/2018	Phase II/III	Completed	Metabolic (Other)	Adrenoleukodystrophy (Adrenomyeloneuropathy/ Schilder-Addison Complex)	CD34 and/or CD133 stem cells	Yes, ex vivo	Lentivirus	Autologous	2013	Great Ormond Street Hospital for Children NHS Foundation Trust, London
Genetix Biotherapeutics Inc	A Study Evaluating the Efficacy and Safety of the Lentiglobin BB305 Drug Product in Participants with Transfusion-dependent β-, Who Do Not Have a β0/β0 Genotype	HGB-207 NCT02906202 EudraCT-2015-004122-33 P/257/2015 IRAS-202539 15324	Phase III	Completed	Haematological	β-Thalassemia	CD34 and/or CD133 stem cells	Yes, ex vivo	Lentivirus	Autologous	2016	University College London Hospitals NHS Foundation Trust, London Great Ormond Street Hospital for Children NHS Foundation Trust, London
Genetix Biotherapeutics Inc	Long-term Follow-up of Participants with Cerebral Adrenoleukodystrophy who were treated with Lenti-D Drug Product	EudraCT-2015-002805-13 06580819.1.0000.0068 3.163.804 CHIL 5109 CTIS2024-513904-33-00 EUCT-2024-513904-33-00 EUCTR2015-002805-13-NL IRAS-129575 LTF-304 NCI-2019-08553 NCT02698579 NL-OMON54368 NL76175.000.21 RBR-72wbbg STUDY00005330 U1111-1249-1366 UKCRN-20304	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 Hospital, London
Genetix Biotherapeutics Inc	A Study Evaluating the Efficacy and Safety of the Lentiglobin BB305 Drug Product in Participants with Transfusion-dependent β- thalassemia	HGB-212 NCT03207009 EudraCT-2016-003611-35 US-1598	Phase III	Completed	Haematological	β-Thalassemia	CD34 and/or CD133 stem cells	Yes, ex vivo	Lentivirus	Autologous	2017	University College London Hospitals NHS Foundation Trust, London
Genetix Biotherapeutics Inc	A Clinical Study to Assess the Efficacy and Safety of Gene Therapy for the Treatment of Cerebral Adrenoleukodystrophy (CALD)	ALD-104 NCT03852498 EudraCT-2018-001145-14 XX-0096 NL68243.000.18 EUCTR2018-001145-14-NL NL-OMON54719	Phase III	Completed	Metabolic (Other)	Adrenoleukodystrophy	CD34 and/or CD133 stem cells	Yes, ex vivo	Lentivirus	Autologous	2019	Great Ormond Street Hospital for Children NHS Foundation Trust, London
Genetix Biotherapeutics Inc	Long-term Follow-up of Subjects With Transfusion-Dependent β-Thalassemia (TDT) Treated With Ex vivo Gene Therapy	LTF-303 EudraCT-2013-002245-11 NCI-2020-03443 EUCT-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

Sponsor	Title	Clinical Database Numbers	Trial Phase	Trial Status	Disease Area	Indication	Cell Type	Gene Modification / Gene Therapy	Type of Vector Used (if applicable)	Autologous/ Allogeneic	Year Trial Started	United Kingdom Site(s)
GenSight Biologics SA	Dose-escalation Study to Evaluate the Safety and Tolerability of GS030 in Subjects with Retinitis Pigmentosa (PIONEER)	NCT03326336 GS030_CLIN_001 EudraCT-2017-002204-27 EUCT-2024-516059-42-00 235242	Phase I/II	In follow-up	Ophthalmology	Retinitis Pigmentosa (Retinitis)	N/A	Yes, <i>in vivo</i>	AAV	N/A	2018	Moorfields Eye Hospital NHS Foundation Trust, London
GenSight Biologics SA	Efficacy & Safety Study of Bilateral IVT Injection of GS010 in LHON Subjects Due to the ND4 Mutation for up to 1 Year (REFLECT)	GS-LHON-CLIN-05 EudraCT-2017-002187-40 1803-1728 US-1728	Phase III	Completed	Ophthalmology	Leber's Hereditary Optic Neuropathy (Leber Optic Atrophy)	N/A	Yes, <i>in vivo</i>	AAV	N/A	2018	Moorfields Eye Hospital NHS Foundation Trust, London
GenSight Biologics SA	RESCUE and REVERSE Long-term Follow-up (RESTORE)	GS-LHON-CLIN-06 EudraCT-2017-002153-11 NCT02652767 RESCUE NCT02652780 REVERSE	Phase III	Completed	Ophthalmology	Leber Hereditary Optic Neuropathy	N/A	Yes, <i>in vivo</i>	AAV	N/A	2018	Moorfields Eye Hospital NHS Foundation Trust, London
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	Haematological	Mantle Cell Lymphoma	T cells	Yes, <i>ex vivo</i>	Retrovirus	Autologous	2023	Decentralised trial utilising telemedicine and registries
Gilead Sciences Inc	Study of KITE-363 or KITE-753 in Participants With Relapsed and/or Refractory B-cell Lymphoma	KT-US-499-0150 EudraCT-2020-000562-41 NL-OMON53464 CTIS2024-511616-24-00 EUCTR2020-000562-41-NL NL81647.000.22 EUCT-2024-511616-24-00	Phase I	Recruiting	Oncology (Haematology)	Relapsed/Refractory Follicular Lymphoma	T cells	Yes, <i>ex vivo</i>	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	NCT05041309 2023-507041-28 (Other Identifier) (OTHER: European Medicines Agency) CTIS2023-507041-28-00 EUCT-2023-507041-28-00 EUCTR2020-005843-21 EudraCT-2020-005843-21 jRCT2013230062 KT-US-982-5968 MOH_2022-08-21_010606 NL-OMON51477 NL79971.000.22 2023-507041-28 (Other Identifier) (OTHER: EU Trial (CTIS) Number)	Phase II	Recruiting	Oncology (Haematology)	Refractory Large B-Cell Lymphoma	T cells	Yes, <i>ex vivo</i>	Retrovirus	Autologous	2021	The Royal Marsden Hospital NHS Foundation Trust, London King's College Hospital NHS Foundation Trust, London
Gilead Sciences Inc	Study to Compare Axicabtagene Ciloleucl With Standard of Care Therapy as First-line Treatment in Participants With High-risk Large B-cell Lymphoma (ZUMA-23)	NCT05605899 KT-US-484-0136 EUCT-2022-501489-24-00 jRCT2023240034	Phase III	Recruiting	Oncology (Other)	Follicular Lymphoma, Diffuse Large B-Cell Lymphoma, Marginal Zone B-cell Lymphoma	T cells	Yes, <i>ex vivo</i>	Retrovirus	Autologous	2023	Addenbrooke's Hospital, Cambridge University Hospitals Southampton NHS Foundation Trust, Southampton

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 Children's Charity	A Trial Investigating the Safety of Thymus-derived Regulatory T Cell Treatment for the Prevention of Cardiac Allograft Vasculopathy in Children Receiving Heart Transplant	1008875 23HL27 IRAS 1008875	Phase I	Recruiting	Cardiovascular	Cardiac Allograft Vasculopathy (CAV)	T cells	No	N/A	Autologous	2024	Great Ormond Street Hospital for Children NHS Foundation Trust, London
Great Ormond Street Hospital for Children NHS Trust	Base Edited CAR T Cells Against AML: Deep Conditioning Ahead of Allogeneic Stem Cell Transplantation (CARAML)	EudraCT- 2021-004311-66 18IC13 ISRCTN14430213 1007053 IRAS 1007053 CPMS 55915	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 Trust	Lentiviral Gene Therapy for X-linked Severe Combined Immunodeficiency	16IC17 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 Trust	Efficacy and Safety of the Cryopreserved Formulation of OTL-101 in Subjects With ADA-SCID	17IC04 EudraCT-2017-001275-23 OTL-101-5 (17IC04) UK-0235	Phase I	Completed	Inflammatory and immune system	Severe Combined Immunodeficiency Due to ADA Deficiency	CD34 and/or CD133 stem cells	Yes, ex vivo	Lentivirus	Autologous	2018	Great Ormond Street Hospital for Children NHS Foundation Trust, London
Great Ormond Street Hospital for Children NHS Trust	TT52CAR19 Therapy for B-Cell Acute Lymphoblastic Leukaemia (B-ALL) (PBLTT52CAR19)	18IC07 2019-003462-40 NCT04557436	Phase I	Completed	Oncology (Haematology)	B-Cell Acute Lymphocytic Leukemia	T cells	Yes, ex vivo	Lentivirus	Allogeneic	2020	Great Ormond Street Hospital for Children NHS Foundation Trust, London
Great Ormond Street Hospital for Children NHS Trust	Base Edited CAR7 T Cells to Treat T Cell Malignancies (Tvt CAR7) (Tvt CAR7)	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 King's College Hospital NHS Foundation Trust, London
Great Ormond Street Hospital for Children NHS 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	Great Ormond Street Hospital for Children NHS Foundation Trust, London

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)
GSK plc	Adoptive Cell Therapy Long-term Follow-up (LTFU) Study	ADP-0000-002 208750 17276 EudraCT-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 ADP Investigational Site, Manchester The Royal Marsden Hospital NHS Foundation Trust, Sutton
GSK plc	Pilot Immunotherapy Study with Letetresgene Autoleucel (Lete-cel, GSK3377794)T-cells in New York Esophageal Squamous Cell Carcinoma-1 (NY-ESO-1)/ LAGE-1a-positive Advanced Non-small Cell Lung Cancer (NSCLC) Either Alone or in Combination with Pembrolizumab	NCT03709706 208471 NCI-2018-03721 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	University College London Hospitals NHS Foundation Trust, London The Christie NHS Foundation Trust, Manchester Guy's and St Thomas' NHS Foundation Trust, London
Guy's and St Thomas' NHS Foundation Trust	Treg Immunotherapy in Crohn's Disease (TRIBUTE)	EudraCT-2017-000170-11 NCT03185000	Phase I	Completed	Gastrointestinal (Other)	Crohn's Disease	T cells	No	N/A	Autologous	2022	Guy's Hospital, Guy's and St Thomas' NHS Foundation Trust, London
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 EudraCT-2017-003712-39 CPPY988A12101	Phase I/II	Terminated	Ophthalmology	Age-related Macular Degeneration (AMD)	N/A	Yes, in vivo	AAV	N/A	2018	Southampton General Hospital, Southampton, London Vision Clinic, London Moorfields Eye Hospital NHS Foundation Trust, London Sunderland Eye Infirmary, South Tyneside and Sunderland NHS Foundation Trust, Sunderland Manchester Eye Hospital, Manchester Bristol Eye Hospital, University Hospitals Bristol and Weston NHS Foundation Trust, 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 NCT04437368 CPPY988A12202 NL71665.000.19 EUCTR2019-003421-22-NL EudraCT-2019-003421-22	Phase II	Terminated	Ophthalmology	Age-related Macular Degeneration (AMD)	N/A	Yes, in vivo	AAV	N/A	2020	Sunderland Eye Infirmary, South Tyneside and Sunderland NHS Foundation Trust, Sunderland Bristol Eye Hospital, University Hospitals Bristol and Weston NHS Foundation Trust, Bristol St Paul's Eye Unit, Liverpool University Hospitals NHS Foundation Trust, Liverpool Sheffield Teaching Hospitals NHS Foundation Trust, Sheffield Moorfields Eye Hospital NHS Foundation Trust, London
Gyroscope Therapeutics Holdings Plc	HORIZON : A Phase II Study to Evaluate the Safety and Efficacy of Two Doses of GT005	NCT04566445 GT005-03 EudraCT-2020-002431-30 CPPY988A12201	Phase II	Terminated	Ophthalmology	Age-related Macular Degeneration (AMD)	N/A	Yes, in vivo	AAV	N/A	2020	Sunderland Eye Infirmary, South Tyneside and Sunderland NHS Foundation Trust, Sunderland Moorfields Eye Hospital NHS Foundation Trust, London John Radcliff Hospital, Oxford Retina Clinic, London
Holostem Terapie Avanzate SRL	long-term safety and efficacy follow-up study after Autologous Cultivated Limbal Stem Cells Transplantation (ACL SCT) for restoration of corneal epithelium in patients with limbal stem cell deficiency due to ocular burns (HOLOCORE-FU)	IRAS ID 184635 EudraCT-2015-001344-11 EUPAS10043	Phase IV	Recruiting	Ophthalmology	Limbal Stem Cell Deficiency	Corneal	No	N/A	Autologous	2016	Newcastle Upon Tyne Hospitals NHS Foundation Trust, Newcastle Upon Tyne Moorfields Eye Hospital NHS Foundation Trust, London

Sponsor	Title	Clinical Database Numbers	Trial Phase	Trial Status	Disease Area	Indication	Cell Type	Gene Modification / Gene Therapy	Type of Vector Used (if applicable)	Autologous/ Allogeneic	Year Trial Started	United Kingdom Site(s)
Holostem Terapie Avanzate SRL	Follow-up Study After ACLSCT for Restoration of Corneal Epithelium in Patients with LSCD Due to Ocular Burns (HOLOCORE-FU)	CCD-GPLSCD01-03-FU EudraCT-2015-001344-11 P/199/2012 IRAS-249754	Phase IV	Completed	Ophthalmology	Limbal Stem Cell Deficiency	Corneal	No	N/A	Autologous	2017	Newcastle Upon Tyne Hospitals NHS Foundation Trust, Newcastle Upon Tyne Moorfields Eye Hospital NHS Foundation Trust, London Nottingham University Hospitals NHS Trust, Nottingham
iECURE Inc	An Open-label Study to Investigate ECUR-506 in Male Babies Less than 9 Months of Age with Neonatal Onset OTC Deficiency	NCT06255782 ECUR-506-OTC-101 ISRCTN10957794 IRAS 1008261 1008261 CPMS 57410 EUCT-2023-506180-34-01	Phase I/II/III	Recruiting	Metabolic (Other)	Urea Cycle Disorders, Ornithine Transcarbamylase Deficiency	N/A	Yes, <i>in vivo</i>	AAV	N/A	2024	Great Ormond Street Hospital for Children NHS Foundation Trust, London Newcastle Upon Tyne Hospitals NHS Foundation Trust, Newcastle Upon Tyne
Immune Bio Inc	A Phase I Open-label Dose Escalation Study of Intravenous Inkmmune in Patients with MDS or AML (Laurel)	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 Southampton Hospital NHS Foundation Trust, Southampton
Innovacell Co Ltd	Skeletal Muscle-derived Cell Implantation for Treatment of Fecal Incontinence (Fidelia)	IC-01-02-5-009 EudraCT-2021-001376-42 2024-518243-38-00 EUCT-2024-518243-38-00 298744 jRCT2033230027 IRAS 298744 FI-3	Phase III	Recruiting	Gastrointestinal (Other)	Fecal Incontinence	Skeletal Muscle	No	N/A	Autologous	2022	St Mary's Hospital, London
Instil Bio Inc	ITIL-168 in Advanced Melanoma (DELTA-1)	EudraCT-2020-003862-37 NCT05050006 IRB-21-273 21-000200 20-698 ITIL-168-101	Phase II	Terminated	Oncology (Other)	Advanced Melanoma	T cells	No	N/A	Autologous	2021	Addenbrooke's Hospital, Cambridge
Intellia Therapeutics Inc	Study to Evaluate Safety, Tolerability, Pharmacokinetics, and Pharmacodynamics of NTLA-2001 in Patients With Hereditary Transthyretin Amyloidosis With Polyneuropathy (ATTRv-PN) and Patients With Transthyretin Amyloidosis-Related Cardiomyopathy (ATTR-CM)	NCT04601051 ITL-2001-CL-001 EudraCT-2020-002034-32 EUCT-2024-511170-69-00	Phase I	In follow-up	Metabolic (Other)	Hereditary Transthyretin Amyloidosis With Polyneuropathy (ATTRv-PN)	N/A	Yes, <i>in vivo</i>	Non-viral	N/A	2020	Richmond Pharmacology Ltd, London
Intellia Therapeutics Inc	Study Investigating NTLA-5001 in Subjects with Acute Myeloid Leukemia	NCT05066165 ITL-5001-CL-001	Phase I/II	Terminated	Oncology (Haematology)	Acute Myelocytic Leukemia (AML)	T cells	Yes, <i>ex vivo</i>	Unknown	Allogeneic	2021	Research Site 10, Leeds Research Site 8, London Research Site 9, London Research Site 7, Manchester

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	NTLA-2002 in Adults with Hereditary Angioedema (HAE) (NTLA-2002)	NCT05120830 ITL-2002-CL-001 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, <i>in vivo</i>	Non-viral	N/A	2021	Cambridge University Hospitals NHS Foundation Trust, 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 U1111-1291-3138 202400788 JRCT2053250023	Phase III	In follow-up	Cardiovascular	Amyloidosis, Congestive Heart Failure (Heart Failure), Familial Amyloid Cardiomyopathy, Amyloid Cardiomyopathy	N/A	Yes, <i>in vivo</i>	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
Intellia Therapeutics Inc	HAELO: A Phase III Study to Evaluate NTLA-2002 in Participants With Hereditary Angioedema (HAE)	ITL-2002-CL-301 NCT06634420 DOH-27-022025-4888 EUCT-2024-515741-42-00	Phase III	In follow-up	Inflammatory and Immune System	Hereditary Angioedema (HAE) (C1 Esterase Inhibitor [C1-INH] Deficiency)	N/A	Yes, <i>in vivo</i>	Non-viral	N/A	2025	Cambridge University Hospitals NHS Foundation Trust, Cambridge
Intellia Therapeutics Inc	Long-Term Follow-Up (LTFU) of Subjects Treated with NTLA 2002	NCT06262399 ITL-2002-CL-999 EudraCT-2022-003778-22 EUCT-2023-507956-56-00	Phase I/II	Recruiting	Genetic Disorders	Hereditary Angioedema Deficiency)	N/A	Yes, <i>in vivo</i>	Non-viral	N/A	2024	Addenbrooke's Hospital, Cambridge
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 SNCTP000002819	Phase II	Completed	Oncology (Other)	Metastatic Melanoma	T cells	No	N/A	Autologous	2015	The Royal Marsden Hospital 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	16819 18959 20161142 2017-010 2017-02024 2017-0505 2024-510634-41-00 BASEC2017-02024 C-145-04 CDR789471 CTIS2024-510634-41-00 EUCT-2024-510634-41-00 EUCTR2016-003447-11 EudraCT-2016-003447-11 IND 16819 J17111 NCI-2017-01051 NCT03108495 NL-OMON53011 NL62470.000.17 P 38016 SNCTP000002862	Phase II	Terminated	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

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	EUCT-2018-001608-12 BASEC2018-01915 EUCT-2024-510779-39-00 IOV-COM-202 NCI-2018-01950 SNCTP000003376	Phase II	Recruiting	Oncology (Other)	Metastatic Melanoma	T cells	No	N/A	Autologous	2019	Guy's Hospital, Guy's and St Thomas' NHS Foundation Trust, London The Royal Marsden Hospital NHS Foundation Trust, London Bristol Haematology and Oncology Centre, Bristol
Iovance Biotherapeutics Inc	Autologous LN-145 in Patients With Metastatic Non-Small-Cell Lung Cancer	IOV-LUN-202 NCT04614103 EudraCT-2020-003629-45 NCI-2020-11568 LUN-202 EUCTR2020-003629-45 2024-510778-26-00 CTIS2024-510778-26-00 NL76099.000.20 NL-OMON52190 EUCT-2024-510778-26-00 IRAS-292831 21-217 RG1121422 292831	Phase II	Recruiting	Oncology (Other)	Non Small Cell Lung Cancer	T cells	No	N/A	Autologous	2021	The Royal Marsden Hospital NHS Foundation Trust, London Guy's and St Thomas' NHS Foundation Trust, London Sarah Cannon Research Institute, London The Christie NHS Foundation Trust, Manchester University College London Hospitals 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 202500015 EUCT-2022-503140-41-00 HumRes67474 CTIS2022-503140-41-00	Phase III	Recruiting	Oncology (Other)	Metastatic Melanoma	T cells	No	N/A	Autologous	2023	University Hospital Birmingham NHS Foundation Trust, Birmingham Cambridge University Hospitals NHS Foundation Trust, Cambridge University College London Hospitals NHS Foundation Trust, London TThe Royal Marsden Hospital NHS Foundation Trust, Sutton King's College Hospital NHS Foundation Trust, London The Christie NHS Foundation Trust, Manchester Oxford University Hospitals NHS Foundation Trust, Oxford The University Hospital Southampton NHS Foundation Trust, Southampton
Ixaka Ltd (Inactive)	The Efficacy and Safety of REX-001 to Treat Ischemic Rest Pain in Subjects with CLI Rutherford Category 4 and DM	NCT03111238 REX-001-004 NL57337.000.16 EUCTR2016-000240-34-NL NL-OMON47803	Phase III	Terminated	Cardiovascular	Cardiovascular Disease, Peripheral Arterial Disease (PAD)/ Peripheral Vascular Disease (PVD), Type 1 Diabetes (Juvenile Diabetes), Type 2 Diabetes, Critical Limb Ischemia	Bone marrow mononuclear cells	No	N/A	Autologous	2017	Addenbrooke's Hospital, Cambridge University Hospital of Wales, Cardiff Freeman Hospital, Newcastle Upon Tyne Rexgenero Limited, Brighton University Hospital of Wales, Cardiff
Ixaka Ltd (Inactive)	The Efficacy and Safety of REX-001 to Treat Ischemic Ulcers in Subjects with CLI Rutherford Category 5 and DM	EudraCT-2016-003980-21 IRAS 219960 REX-001-005	Phase III	Terminated	Cardiovascular	Critical Limb Ischemia in Patients with Diabetes Mellitus	Bone marrow mononuclear cells	No	N/A	Autologous	2017	University Hospital of Wales, Cardiff Birmingham University Hospital, Birmingham Addenbrooke's Hospital, Cambridge Freeman Hospital, Newcastle Upon Tyne
Johnson & Johnson	Long-term Follow-up Gene Therapy Study for RPGR- XLRP	EudraCT-2018-000425-31 MGT010 NCT04312672 UK-0230	Phase I/II	In follow-up	Ophthalmology	X-Linked Retinitis Pigmentosa	N/A	Yes, <i>in vivo</i>	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 CT1175 EUCT-2023-506588-32-00 EudraCT-2019-001413-16 JRCT1080225184 MMY3002 MOH_2020-06-08_008780 NCI-2020-05552 OSU-20005 JapicCTI-205280	Phase III	In follow-up	Oncology (Haematology)	Multiple Myeloma	T cells	Yes, <i>ex vivo</i>	Lentivirus	Autologous	2020	Queen Elizabeth Hospital, Birmingham University Hospitals Birmingham NHS Foundation Trust, Birmingham Bristol Haematology and Oncology Centre, University Hospitals Bristol and Weston NHS Foundation Trust, Bristol University College London Hospitals NHS Foundation Trust, London King's College Hospital NHS Foundation Trust, London Freeman Hospital, Newcastle Upon Tyne Hospitals NHS Foundation Trust, Newcastle Upon Tyne

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	Gene Therapy Trial for the Treatment of X-linked Retinitis Pigmentosa Associated With Variants in the RPGR Gene	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 HumRes61758	Phase III	Completed	Ophthalmology	X-Linked Retinitis Pigmentosa	N/A	Yes, <i>in vivo</i>	AAV	N/A	2020	NHS Lothian, Edinburgh Gartnavel General Hospital, Glasgow Moorfields Eye Hospital NHS Foundation Trust, London St James's University Hospital, Leeds
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 EudraCT-2020-002255-37 EUCTR2020-002255-37 2021-02178 SNCTP000004847 BASEC2021-02178 2020-002255-37-IE EUCT 2024-511411-25-00 IND 17,866	Phase III	In follow-up	Ophthalmology	X-Linked Retinitis Pigmentosa	N/A	Yes, <i>in vivo</i>	AAV	N/A	2020	NHS Lothian, Edinburgh Gartnavel General Hospital, Glasgow St James's University Hospital, Leeds Moorfields Eye Hospital NHS Foundation Trust, London
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 EUCT 2023-505850-16-00 CTIS2023-505850-16-00	Phase III	In follow-up	Oncology (Haematology)	Multiple Myeloma	T cells	Yes, <i>ex vivo</i>	Lentivirus	Autologous	2021	University Hospitals Birmingham NHS Trust, Birmingham Bristol Royal Infirmary, Bristol Leeds Teaching Hospitals NHS Trust, Leeds University College Hospitals NHS Foundation Trust, London King's College Hospital NHS Foundation Trust, London Manchester Royal Infirmary, Manchester The Royal Marsden Hospital NHS Foundation Trust, Sutton
Johnson & Johnson	A Study of JNJ-90014496 in Participants with B-Cell Non-Hodgkin Lymphoma	1008775 EudraCT-2023-506267-33 202400725 90014496LYM1001 CPMS 58328 EUCT-2023-506267-33-00 IRAS 1008775 ISRCTN15038320 NCT05421663	Phase I/II	Recruiting	Oncology (Haematology)	B-Cell non-Hodgkin lymphoma	T cells	Yes, <i>ex vivo</i>	Lentivirus	Autologous	2022	University College London Hospitals NHS Foundation Trust, London The Christie NHS Foundation Trust, Manchester
Johnson & Johnson	A Study of JNJ-90009530 in Relapsed or Refractory B-Cell Non-Hodgkin Lymphoma (<i>r/r</i> B-NHL)	90009530LYM1001 CPMS 57721 EudraCT-2023-506259-97 GDC30033094 GDCT0486773 IRAS 1009316 ISRCTN17666983	Phase I	Recruiting	Oncology (Haematology)	B-Cell non-Hodgkin lymphoma	T cells	Yes, <i>ex vivo</i>	Lentivirus	Autologous	2023	University College London Hospitals 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 ISRCTN38641780 1006234 IRAS 1006234 CPMS 53494 BASEC2023-00555 EUCTR2022-500746-16 SNCTP000005519 2023-00555 CTIS2022-500746-16-00 EUCT-2022-500746-16-00	Phase II	In follow-up	Ophthalmology	Dry (Atrophic) Macular Degeneration	N/A	Yes, <i>in vivo</i>	AAV	N/A	2023	Bristol Eye Hospital, Bristol Moorfields Eye Hospital NHS Foundation Trust, London Central Middlesex Hospital, London 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

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 Long-term Extension Study of JNJ-81201887 (AAV/CAGsCD59) Parent Studies in Participants with Geographic Atrophy (GA) Secondary to Age-related Macular Degeneration (AMD)	1010481 81201887MDG3002 CTIS2022-500747-21-00 CTR20251128 ISRCTN11083692 EUCT-2022-500747-21-00 EDMS-RIM-606747 HumRes67177	Phase II	Recruiting	Ophthalmology	Age-related Macular Degeneration (AMD)	N/A	Yes, <i>in vivo</i>	AAV	N/A	2024	Oxford Eye Hospital, Oxford Bristol Eye Hospital, Bristol Retina Clinic, London
Johnson & Johnson	A Follow-on Study for Second-eye Treatment for Participants Previously Treated with Gene Therapy for X-linked Retinitis Pigmentosa (XLRP)	1010255 74765340RPG2001 CPMS 62454 ISRCTN57691385	Phase II	Recruiting	Ophthalmology	X-Linked Retinitis Pigmentosa	N/A	Yes, <i>in vivo</i>	AAV	N/A	2024	Moorfields Eye Hospital NHS Foundation Trust, London
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, Progressive Forms of Multiple Sclerosis or Refractory Myasthenia Gravis (MG) (Breakfree-2)	CA061-1006 NCT06220201 U1111-1296-8579 EUCT-2023-507820-22-00	Phase I	Recruiting	Neurological	Multiple Sclerosis, Myasthenia Gravis, Primary Progressive Multiple Sclerosis (PPMS), Relapsing Multiple Sclerosis (RMS), Secondary Progressive Multiple Sclerosis (SPMS)	T cells	Yes, <i>ex vivo</i>	Lentivirus	Autologous	2024	Barts Health NHS Trust, London Manchester Royal Infirmary, Manchester University College London Hospitals NHS Foundation Trust, London Salford Royal Hospital, Salford
Juno Therapeutics Inc	A Study of CC-97540 (BMS-986353), CD19-Targeted NEX-T CAR T Cells, in Participants with Active SLE Despite Immunosuppressants (Breakfree-SLE)	CA061-1011 EUCT-2024-519278-37-00 2024-519278-37-00 U1111-1315-1600 CTIS-2024-519278-37-00	Phase II	Recruiting	Inflammatory and Immune System	Systemic Lupus Erthematosus/Lupus Nephritis	T cells	Yes, <i>ex vivo</i>	Lentivirus	Autologous	2025	King's College Hospital NHS Foundation Trust, London University College London Hospitals NHS Foundation Trust, London
Kalaris Therapeutics Inc	Study to Evaluate Viralm-M (ALVR105) for the Treatment of Virus-Associated Hemorrhagic Cystitis (HC)	NCT04390113 AVM-003-HC NCI-2020-04433 EudraCT-2020-000722-26 202100534 GDCT0410260 GDC30026557	Phase III	Terminated	Renal and Urogenital	Herpesviridae Infections, Hematopoietic Stem Cell Transplantation, Viral Infections, Primary Immune Deficiency (PID), Cytomegalovirus (HHV-5) Infections, Adenoviridae Infections, Epstein-Barr Virus (HHV-4) Infections, Hemorrhagic Cystitis (Haemorrhagic Cystitis), Polyomavirus Infections, Pyelonephritis	T cells	No	N/A	Allogeneic	2021	Queen Elizabeth University Hospital, Birmingham Nottingham University Hospitals NHS Trust, Nottingham University Hospitals Bristol and Weston NHS Foundation Trust, Bristol Great Ormond Street Hospital for Children NHS Foundation Trust, London Hammersmith Hospital, London The Royal Marsden Hospital NHS Foundation Trust, London University College London Hospitals NHS Foundation Trust, London
Kalaris Therapeutics 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 EudraCT-2021-003450-22	Phase III	Terminated	Infectious Disease	Adenoviridae Infections	T cells	No	N/A	Allogeneic	2022	Sheffield Children's Hospital, 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
Kalaris Therapeutics Inc	Study of Posoleucel (ALVR105, Viralm-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	Multi-virus prevention of ADV, BKV, CMV, EBV, HHV-6, and JCV infection in Allogeneic Hematopoietic Transplant Subjects	T cells	No	N/A	Allogeneic	2022	Bristol Haematology and Oncology Centre, London University Hospitals Bristol and Weston NHS Foundation Trust, Bristol Addenbrooke's Hospital, Cambridge Queen Elizabeth University Hospital, Glasgow Great Ormond Street Hospital for Children NHS Foundation Trust, London Royal Hospital for Children, Glasgow St Mary's Hospital, London Hammersmith Hospital, London The Royal Marsden Hospital NHS Foundation Trust, London Royal Manchester Children's Hospital, Manchester Bristol Royal Hospital for Children, Bristol

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)
Kalaris Therapeutics Inc	Global Registry for Long-term Follow-up of Patients Participating in Clinical Trials with Posoleucel (ALVR105)	2022-000763-45 P-105-401 IRAS 1005578 1005578 ISRCTN31439492 CPMS 51835 P-105-401/CON 4690/0012	Phase IV	Recruiting	Infectious Disease	Treatment for 6 viral pathogens potentially transmitted through transplantation	T cells	No	N/A	Allogeneic	2021	Addenbrooke's Hospital, Cambridge Bristol Haematology and Oncology Centre, Bristol University College London Hospitals NHS Foundation Trust, London Birmingham Childrens Hospital NHS Foundation Trust, Birmingham King's College Hospital NHS Foundation Trust, London Royal Hospital for Children, Bristol Sheffield Childrens Hospital, Sheffield Royal Manchester Children's Hospital, Manchester Great Ormond Street Hospital for Children NHS Foundation Trust, London Nottingham University Hospitals NHS Trust, Nottingham The Royal Marsden Hospital NHS Foundation Trust, London Queen Elizabeth Hospital, Birmingham St Mary's Hospital, London Royal Hospital for Children, Glasgow
Keele University	Autologous Chondrocyte Transplantation/Implantation Versus Existing Treatments	G0200055	Phase III	Completed	Musculoskeletal	Musculoskeletal Disorders	Chondrocytes	No	N/A	Autologous	2004	Frimley Park Hospital, Surrey University Hospitals Sussex NHS Foundation Trust, Sussex Great Western Hospitals NHS Foundation Trust, Swindon Oxford University Hospitals NHS Foundation Trust, Oxford Western General Hospital, Weston-super-Mare Princess Elizabeth Orthopaedic Centre, Exeter The Robert Jones and Agnes Hunt Orthopaedic Hospital NHS Trust, Shropshire North Staffordshire Royal Infirmary, Staffordshire New Cross Hospital, Wolverhampton West Midlands Hospital, Birmingham The Royal Orthopaedic Hospital NHS Foundation Trust, Birmingham Warrington and Halton Hospitals NHS Foundation Trust, Warrington
Kiadis Pharma NV	Safety and Efficacy of ATIR101 as Adjunctive Treatment to Blood Stem Cell Transplantation from a Haploidentical Family Donor Compared to Post-transplant Cyclophosphamide in Patients with Blood Cancer (HATCY)	CR-AIR-009 NCT02999854 EudraCT-2016-004672-21 NCI-2017-01213 CDR789821 MOH_2019-03-24_006041 NL61672.000.17 EUCTR2016-004672-21-NL NL-OMON48941	Phase III	Terminated	Oncology (Haematology)	Haematologic malignancies (AML, ALL, or MDS)	T cells	No	N/A	Allogeneic	2017	Heartlands Hospital, Birmingham St James's University Hospital, Leeds Royal Liverpool University Hospital, Liverpool Hammersmith Hospital, London Manchester Royal Infirmary, Manchester
King's College Hospital NHS Foundation Trust	A Large, Prospective Real-world Cohort Study of Axicabtagene ciloleucel or Tisagenlecleucel on Safety and Efficacy in Subjects with Relapsed/refractory High-grade Lymphoma		Phase IV	In follow-up	Oncology (Haematology)	Lymphoma Diffuse Large B-Cell Lymphoma	T cells	Yes, ex vivo	Lentivirus	Autologous	2018	King's College Hospital NHS Foundation Trust, London
King's College Hospital NHS Foundation Trust	Clinical Study of CD19 Car-T Therapy in Subjects with Large B- cell Lymphoma		Phase IV	Completed	Oncology (Other)	Diffuse Large B-Cell Lymphoma Primary Mediastinal B-Cell Lymphoma	T cells	Yes, ex vivo	Retrovirus	Autologous	1900	Freeman Hospital, Newcastle Upon Tyne
King's College Hospital NHS Foundation Trust	Hepatocyte Microbeads for Acute Liver Failure (HELP)	3571 2019-000316-29 199623 ISRCTN14716227 CPMS 52663 MR/V038583/1	Phase I/II	Recruiting	Gastrointestinal (Liver)	Acute Liver Failure	Mesenchymal stem/stromal cells	No	N/A	Allogeneic	2022	King's College Hospital NHS Foundation Trust, London Bupa Foundation, London
King's College Hospital NHS Foundation Trust	Autologous Tregs for Aplastic Anaemia (TIARA)	2021-000082-33 294362	Phase I	Recruiting	Haematological	Aplastic Anemia	T cells	No	N/A	Autologous	2022	King's College Hospital NHS Foundation Trust, London

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)
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	Completed	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 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 KTE-C19-107	Phase III	Completed	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 Hospital NHS Foundation Trust, Sutton
Kite Pharma Inc	Study of Brexucabtagene Autoleucel (KTE-X19) in Participants With Relapsed/Refractory Mantle Cell Lymphoma (Cohort 3) (ZUMA-2)	KTE-C19-102 (Cohort 3) EudraCT- 2015-005008-27 EUCT-2023-506641-35-00	Phase II	Completed	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 EudraCT-2021-003260-28 jRCT2063230096 EUCT-2024-511594-30-00	Phase III	In follow-up	Oncology (Other)	Relapsed/Refractory Follicular Lymphoma, Follicular Lymphoma	T cells	Yes, ex vivo	Retrovirus	Autologous	2022	University Hospitals Birmingham NHS Foundation Trust, Birmingham Cambridge University Hospitals NHS Foundation Trust, Cambridge King's College Hospital NHS Foundation Trust, London The Christie NHS Foundation Trust, Manchester The University Hospital Southampton NHS Foundation Trust, Southampton University College London Hospitals NHS Foundation Trust, London Oxford University Hospitals NHS Foundation Trust, Oxford The Royal Marsden Hospital NHS Foundation Trust, Sutton
Kite Pharma Inc	A Study Comparing Anitocabtagene Autoleucel to Standard of Care Therapy in Participants with Relapsed/ Refractory Multiple Myeloma (IMMagine-3)	EUCT-2024-511188-26-00 jRCT2043240170 KT-US-679-0788 NCT06413498	Phase III	Recruiting	Oncology (Other)	Multiple Myeloma (Kahler Disease), Refractory Multiple Myeloma, Relapsed Multiple Myeloma	T cells	Yes, ex vivo	Lentivirus	Autologous	2024	Freeman Hospital, Newcastle Upon Tyne Bristol Haematology and Oncology Centre, University Hospitals Bristol and Weston NHS Foundation Trust, Bristol King's College Hospital, University College London Hospitals NHS Foundation Trust, London University Hospital of Wales, Cardiff St James's University Hospital, Leeds Teaching Hospitals NHS Trust, Leeds
Kuopio University Hospital	Adenovirus Vascular Endothelial Growth Factor D (AdvVEGF-D) Therapy for Treatment of Refractory Angina Pectoris (ReGenHeart)	KUH5101111 2017-000789-31 RGH201 731532 2024-512255-20	Phase II	Completed	Cardiovascular	Refractory Angina Pectoris	N/A	Yes, in vivo	Adenovirus	N/A	2019	Queen Mary University of London, London

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)
Laboratoires Pierre Fabre SA	A Phase III Study of Tabelecleucel for Participants with Epstein-barr Virus-associated Post-transplant Lymphoproliferative Disease after Failure with Rituximab or Rituximab and Chemotherapy (ALLELE)	1708018515 20005 20161025 201612068 2017-002949-30 2017-0769 2017-0771 2024-516622-57-00 ATA129-EBV-301 ATA129-EBV-302/ F60085DL302 GDC20006702 GDCT0253980 NCI-2018-00043 NCI-2018-00293 NCT03392142 NCT03394365 OSU-18035	Phase III	Recruiting	Oncology (Haematology)	Epstein-Barr Virus-Associated Post-Transplant Lymphoproliferative Disease	T cells	No	N/A	Allogeneic	2017	Imperial College Healthcare NHS Trust, London University Hospitals Birmingham NHS Foundation Trust, Birmingham King's College Hospital NHS Foundation Trust, London
Laboratoires Pierre Fabre SA	A Study to Evaluate Tabelecleucel in Participants with Epstein-barr Virus (EBV)-Associated Diseases	205 ATA129-EBV-205/ F60085DL205 EudraCT-2020-000177-25 NCI-2020-07698 NCT04554914 2024-516623-14-00 (EU Trial (CTIS) 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 Foundation Trust, London University Hospitals Birmingham NHS Foundation Trust, Birmingham
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 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 The Christie NHS Foundation Trust, Manchester
Leucid Bio Ltd	LEU01101: Safety and Preliminary Efficacy of LEU011 in Solid Tumours. (AERIAL)	NCT06193902 LEU01101	Phase I/II	Recruiting	Oncology (Other)	Relapsed/Refractory Solid Tumours	T cells	Yes, ex vivo	Unknown	Autologous	2023	Guy's and St Thomas' NHS Foundation Trust, London The Christie NHS Foundation Trust, Manchester
Lysogene SAS	A Safety and Efficacy Study of LYS-GM101 Gene Therapy in Patients with GM1 Gangliosidosis	NCT04273269 P1-GM-101 2019-004949-32 19440	Phase I/II	Terminated	Metabolic (Other)	GM1 Gangliosidosis	N/A	Yes, in vivo	AAV	N/A	2021	Royal Manchester Children's Hospital, Central Manchester University Hospitals NHS Foundation Trust, Manchester
Lysogene SAS	Study of AAVrh10-h.SGSH Gene Therapy in Patients with Mucopolysaccharidosis Type IIIA (MPS IIIA) (AAVance)	NCT03612869 P4-SAF-302 XX-0066 2018-000195-15 NL-OMON52445 NL65877.000.18	Phase II/III	Completed	Metabolic (Other)	Mucopolysaccharidosis Type IIIA	N/A	Yes, in vivo	AAV	N/A	2018	Great Ormond Street Hospital for Children NHS Foundation Trust, London
Mario Negri Institute for Pharmacological Research	NEPHSTROM for Diabetic Kidney Disease (NEPHSTROM)	634086 H2020-PHC-2014 PHC-13-2014 NUIG 089-15 2016-000661-23 GDCT0270832 GDC30011730	Phase I/II	Terminated	Renal and Urogenital	Diabetic Kidney Disease (Type 2 Diabetes and Chronic Kidney Disease)	Mesenchymal stem/stromal cells	No	N/A	Allogeneic	2017	Queen Elizabeth Hospital, Birmingham Belfast City Hospital, Belfast

Sponsor	Title	Clinical Database Numbers	Trial Phase	Trial Status	Disease Area	Indication	Cell Type	Gene Modification / Gene Therapy	Type of Vector Used (if applicable)	Autologous/ Allogeneic	Year Trial Started	United Kingdom Site(s)
MeiraGTx Holdings Plc	Gene Therapy for X-linked Retinitis Pigmentosa (XLRP) - Retinitis Pigmentosa GTPase Regulator (RPGR)	NCT03252847 MGT009 EudraCT-2016-003967-21 17/LO/0020 IRAS218294 US-1755 US-1740	Phase I/II	Completed	Ophthalmology	X-Linked Retinitis Pigmentosa	N/A	Yes, <i>in vivo</i>	AAV	N/A	2017	Moorfields Eye Hospital NHS Foundation Trust, London Leeds Teaching Hospitals NHS Trust, Leeds
MeiraGTx Holdings Plc	Long-Term Follow-up Gene Therapy Study for Leber Congenital Amaurosis OPTIRPE65 (Retinal Dystrophy Associated with Defects in RPE65)	MGT004 EudraCT-2016-000898-20 BAIJ1014 IRAS-205081	Phase I/II	Completed	Ophthalmology	Leber Congenital Amaurosis (LCA)	N/A	Yes, <i>in vivo</i>	AAV	N/A	2016	Moorfields Eye Hospital NHS Foundation Trust, London
MeiraGTx Holdings Plc	Long-term Follow-up Gene Therapy Study for Achromatopsia CNGB3 and CNGA3	MGT007 EudraCT-2016-003856-59 IRAS-216171 UK-0232 216171	Phase I/II	Completed	Ophthalmology	Achromatopsia	N/A	Yes, <i>in vivo</i>	AAV	N/A	2017	Moorfields Eye Hospital NHS Foundation Trust, London
MeiraGTx Holdings Plc	Gene Therapy for Achromatopsia (CNGA3) (CNGA3)	MGT012 EudraCT-2018-003431-29 GDC30020452 GDCT0351552	Phase I/II	Completed	Ophthalmology	Achromatopsia	N/A	Yes, <i>in vivo</i>	AAV	N/A	2019	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 NCI-2023-05646 1007453	Phase II	Recruiting	Oral	Xerostomia	N/A	Yes, <i>in vivo</i>	AAV	N/A	2023	Addenbrooke's Hospital, Cambridge Ninewells Hospital and Medical School, Dundee Western General Hospital, London Guy's Hospital, Guy's and St Thomas' NHS Foundation Trust, London Nottingham University Hospitals NHS Trust
Mesoblast Ltd	Safety Study of Allogeneic Mesenchymal Precursor Cell Infusion in Myocardial Infarction (AMICI)	ANG.AMI-IC001 EudraCT-2010-020497-41 109985 NL36947.000.11 EUCTR2010-020497-41-NL NL-OMON45102	Phase II	Completed	Cardiovascular	Myocardial Infarction	Mesenchymal stem/stromal cells	No	N/A	Allogeneic	2013	Royal Brompton & Harefield NHS Foundation Trust, University of Glasgow Newcastle Upon Tyne Hospitals NHS Foundation Trust, Newcastle Upon Tyne
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, <i>in vivo</i>	Non-viral	N/A	2016	University Hospitals Birmingham NHS Foundation Trust, Birmingham Cambridge University Hospitals NHS Trust, Cambridge The Beatson West of Scotland Cancer Centre, Glasgow Clatterbridge Cancer Centre NHS Foundation Trust, Liverpool Guy's and St. Thomas' NHS Foundation Trust, London Imperial College Healthcare NHS Trust, London University College London Hospitals NHS Foundation Trust, London Newcastle Upon Tyne Hospitals NHS Foundation Trust, Newcastle Upon Tyne

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	Open-label Study of mRNA-3927 in Participants with Propionic Acidemia	EUCT-2022-502910-10-00 EudraCT-2019-003529-36 IRAS ID 1003761 JRCT2043240044 mRNA-3927-P101 NCT04159103 CTIS 2022-502910-10-00 JPRN-JRCT2043240044	Phase I/II	Recruiting	Metabolic (Other)	Propionic Acidemia	N/A	Yes, <i>in vivo</i>	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	A Study to Assess Safety, Pharmacokinetics, and Pharmacodynamics of mRNA-3705 in Participants with Isolated Methylmalonic Acidemia	NCT04899310 mRNA-3705-P101 EudraCT-2020-004980-24 2022-502492-32-00 EUCT-2022-502492-32-00 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, <i>in vivo</i>	Non-viral	N/A	2021	Royal Manchester Children's Hospital, Manchester Birmingham Children's Hospital NHS Foundation Trust, Birmingham
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	mRNA-3927-P101-EXT EUCT-2022-502911-12-00 JRCT2043250110	Phase I/II	Recruiting	Metabolic (Other)	Propionic Acidemia	N/A	Yes, <i>in vivo</i>	Non-viral	N/A	2021	University Hospitals Birmingham NHS Foundation Trust, Birmingham Great Ormond Street Hospital for Children NHS Foundation Trust, London Willink Biochemical Genetics Unit, Manchester Birmingham Women's and Children's NHS Foundation Trust, Birmingham
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 mRNA-3705-P101-EXT EUCT-2022-501997-20-00 28037 EudraCT-2021-000446-17	Phase I/II	Recruiting	Metabolic (Other)	Methylmalonic Acidemia	N/A	Yes, <i>in vivo</i>	Non-viral	N/A	2022	Birmingham Children's Hospital NHS Foundation Trust, Birmingham Royal Manchester Children's Hospital, Manchester
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)	9494 EudraCT-2015-002125-19 GDC20005759 IRAS-196188 RECHMPL15_0453	Phase II	Completed	Musculoskeletal	Osteoarthritis (Knee)	Mesenchymal stem/stromal cells	No	N/A	Autologous	2016	Cambridge University Hospitals NHS Foundation Trust, Cambridge
Moorfields Eye Hospital NHS Foundation Trust	A Study of Implantation of Retinal Pigment Epithelium in Subjects with Acute Wet Age Related Macular Degeneration	DACL1010 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)	GDC30028763 CLN5-200	Phase I/II	In follow-up	Neurological	Batten Disease	N/A	Yes, <i>in vivo</i>	AAV	N/A	2022	Great Ormond Street Hospital for Children NHS Foundation Trust, London

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 Females with Rett Syndrome	GDC30032284 RTT-200	Phase III	Recruiting	Neurological	Rett Syndrome	N/A	Yes, <i>in vivo</i>	AAV	N/A	2023	Royal Hospital for Children and Young People, Edinburgh Manchester University NHS Foundation Trust, Manchester
Neurotech Pharmaceuticals Inc	A Study to Determine the Safety and Efficacy of NT-501 in Macular Telangiectasia Type 2 - Protocol A	NCT03316300 EudraCT-2017-003234-82 NTMT-03-A US-1680 18-EI-0055 NTMT-03A	Phase III	Completed	Ophthalmology	Macular Telangiectasia	Retinal	Yes, <i>ex vivo</i>	Non-viral	Allogeneic	2017	Oxford Eye Hospital, Oxford Moorfields Eye Hospital NHS Foundation Trust, London
Neurotech Pharmaceuticals Inc	Phase IV Study: Long-term Safety and Efficacy of NT-501 in MacTel Type 2, Including Sham Procedure Participants	NTMT-04	Phase IV	In planning/set-up	Ophthalmology	Macular Telangiectasia	Retinal	Yes, <i>ex vivo</i>	Plasmid	Allogeneic	2025	Oxford University Hospitals NHS Foundation Trust, Oxford
NHS Blood and Transplant	Recovery and Survival of Stem Cell Originated Red Cells	CPMS: 39643 IRAS: 229563 229563 EudraCT-2017-002178-38	Phase I	Recruiting	Haematological	Blood Transfusions	CD34 and/or CD133 stem cells	No	N/A	Allogeneic	2021	Cambridge Donor Centre, NHS Blood and Transplant, Cambridge NIHR Cambridge Clinical Research Facility, Cambridge
Northwest Biotherapeutics Inc	Study of a Drug [DCVax-L] to Treat Newly Diagnosed GBM Brain Cancer	020221 EudraCT-2011-001977-13 CDR453379 NCRN592 UKCRN-14222 NCI-2010-00822 IRAS-131576	Phase III	Completed	Oncology (Other)	Astrocytoma, Glioblastoma Multiforme (GBM), Glioma, Brain Cancer, Brain Tumor	Dendritic cells	No	N/A	Autologous	2007	Addenbrooke's Hospital, Cambridge King's College Hospital NHS Foundation Trust, London University College London Hospitals NHS Foundation Trust, London Queen Elizabeth Hospital, Birmingham
Novartis AG	CAR-T Long Term Follow Up (LTFU) Study (PAVO)	NCT02445222 CDR774629 NCI-2015-01238 EudraCT-2014-001673-14 STU 032015-068 IRB00011960 OSU-15042 2015-0569 CCTL019A2205B JapicCTI-205095 2014-001673-14-AT CTR20220687 JRCT1080225004 NL-OMON53025 CTL019A2205B (PAVO / CART-LTFU) CTIS2023-508128-37-00 EUCTR2014-001673-14-NL NL56825.000.16 EUCT-2023-508128-37-00	Phase II/III	Recruiting	Oncology (Haematology)	Unspecified B-Cell Lymphomas	B-Cell Leukemia	T cells	Yes, <i>ex vivo</i>	Lentivirus	2016	Novartis Investigative Site, Birmingham Novartis Investigative Site, London
Novartis AG	Single-Dose Gene Replacement Therapy Clinical Trial for Participants With Spinal Muscular Atrophy Type 1 (STRIVE-EU)	NCT03461289 AVXS-101-CL-302 EudraCT-2017-000266-29 XX-0048 CL-302 IND 15699 COAV101A12301	Phase III	Completed	Neuromuscular	Spinal Muscular Atrophy	N/A	Yes, <i>in vivo</i>	AAV	N/A	2018	Great Ormond Street Hospital for Children NHS Foundation Trust, London The John Walton Muscular Dystrophy Research Centre, Newcastle The Great North Children's Hospital, Newcastle

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	Efficacy and Safety of Tisagenlecleucel in Adult Patients with Refractory or Relapsed Follicular Lymphoma (ELARA)	18346 217279 CCTL019E2202 CT1030 CTIS2023-508127-13-00 EUCT-2023-508127-13-00 EUCTR2017-004385-94-NL EudraCT-2017-004385-94 JapicCTI-194610 jRCT1080224541 NCI-2018-03720 NCT03568461 NL-OMON55846 NL66135.000.18 OSU-18248 XX-0063	Phase II	Completed	Oncology (Haematology)	Follicular Lymphoma	T cells	Yes, ex vivo	Lentivirus	Autologous	2018	Queen Elizabeth Hospital NHS Foundation Trust, Birmingham King's College Hospital NHS Foundation Trust, London
Novartis AG	Tisagenlecleucel in Adult Patients with Aggressive B-cell Non-hodgkin Lymphoma (BELINDA)	2023-508343-48-00 256235 BASEC2018-01892 CCTL019H2301 CTR20200561 ES-0048 EUCT-2023-508343-48-00 EudraCT-2016-002966-29 IRAS 256235 IRB18-1014 JapicCTI-194897 jRCT1080224814 NCI-2018-02925 NCT03570892 NL-OMON56315 OSU-18222 SNCTP000003400 STUDY00143907	Phase III	In follow-up	Oncology (Haematology)	Non-Hodgkin Lymphoma	T cells	Yes, ex vivo	Lentivirus	Autologous	2019	Queen Elizabeth Hospital, Birmingham University College Hospital, London
Novartis AG	Pre-symptomatic Study of Intravenous Onasemnogene Apeparnovec-xioi in Spinal Muscular Atrophy (SMA) for Patients with Multiple Copies of SMN2 (SPR1NT)	NCT03505099 AVXS-101-CL-304 EudraCT-2017-004087-35 B/BE/18/BVW6 1802-1718 JapicCTI-184203 XX-0060 MOH_2019-07-15_003587 AVXS-101-CL-304 (COAV101A12303) Study CL-304 jRCT1080224142 NL65636.000.18 EUCTR2017-004087-35-NL NL-OMON46178	Phase III	Completed	Neuromuscular	Spinal Muscular Atrophy	N/A	Yes, in vivo	AAV	N/A	2018	Great Ormond Street Hospital for Children NHS Foundation Trust, London
Novartis AG	Phase II Open Label Trial to Determine Safety & Efficacy of Tisagenlecleucel in Pediatric Non-Hodgkin Lymphoma Patients (BIANCA)	NCT03610724 CCTL019C2202 EudraCT-2017-005019-15 JapicCTI-194781 XX-0073 FP00016184 P/266/2017 jRCT1080224705 NL66860.000.18	Phase II	Completed	Oncology (Haematology)	B-Cell Non-Hodgkin Lymphoma	T cells	Yes, ex vivo	Lentivirus	Autologous	2019	Novartis Investigative Site, London Royal Manchester Children's Hospital, Manchester

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	Study of Efficacy and Safety of Tisagenlecleucel in HR B-ALL EOC MRD Positive Patients (CASSIOPEIA)	EudraCT-2017-002116-14 CCTL019G2201J 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 NL66137.000.18 CTL019G2201J EUCT-2023-508081-15-00	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 Study of Patients Receiving Onasemnogene Apeparvovec-xioi	AVXS-101-LT-002 EudraCT-2019-002611-26 JapicCTI-205305 LT-002 COAV101A12103 JRCT1080225209 205305 EUCT-2024-513086-39-00	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 The Newcastle Upon Tyne Hospitals NHS Foundation Trust, Newcastle Upon Tyne
Novartis AG	Safety and Efficacy of Intravenous OAV101 (AVXS-101) in Pediatric Patients with Spinal Muscular Atrophy (SMA) (SMART)	COAV101A12306 EudraCT-2020-005995-37 14042 EUCTR2020-005995-37 2020-005995-37-FR SNCTP000004764 BASEC2021-01258	Phase III	Completed	Neuromuscular	Spinal Muscular Atrophy	N/A	Yes, in vivo	AAV	N/A	2021	Great North Children's Hospital, Newcastle
Novartis AG	PHE885 CAR-T Therapy in Adult Participants with Relapsed and Refractory Multiple Myeloma	NCT05172596 CPHE885B12201 EudraCT-2021-003747-22 JRCT2013220012 202300592 Euct-2023-507140-37-00 1004417 CTIS2023-507140-37-00	Phase II	Terminated	Oncology (Haematology)	Refractory Multiple Myeloma	T cells	Yes, ex vivo	Lentivirus	Autologous	2022	Novartis Investigative Site, Birmingham Novartis Investigative Site, Glasgow Novartis Investigative Site, Manchester
Novartis AG	Long-term Follow-up of Patients With Spinal Muscular Atrophy Treated With OAV101 in Clinical Trials (SPECTRUM)	COAV101A12308 EudraCT-2021-006781-21 NMRR ID-23-03026-17U JRCT2033230642 CTR20240883 EUCTR2021-006781-21 EUCT-2024-511707-42-00 CTRI/2025/02/080626	Phase III	In follow-up	Neuromuscular	Spinal Muscular Atrophy	N/A	Yes, in vivo	AAV	N/A	2022	Great Ormond Street Hospital for Children NHS Foundation Trust, London Great North Children's Hospital, Newcastle Upon Tyne
Novartis AG	Phase II Study Evaluating Rapcabtagene Autoleucel in Participants with Diffuse Cutaneous Systemic Sclerosis	CYTB323K12201 EUCT-2023-510380-34-00 NCT06655896 U1111-1309-0489 JRCT2013240056 HumRes66918 202500395	Phase II	Recruiting	Inflammatory and immune system	Diffuse Cutaneous Systemic Sclerosis, Systemic Sclerosis (Scleroderma)	T cells	Yes, ex vivo	Unknown	Autologous	2024	Novartis Investigative Site, Sheffield

Sponsor	Title	Clinical Database Numbers	Trial Phase	Trial Status	Disease Area	Indication	Cell Type	Gene Modification / Gene Therapy	Type of Vector Used (if applicable)	Autologous/ Allogeneic	Year Trial Started	United Kingdom Site(s)
Novartis AG	A Study of Rapcabtagene Autoleucel in Active, Refractory Systemic Lupus Erythematosus (SLE) or Lupus Nephritis (LN) Patients (AUTOGRAPH - SLE/LN)	CYTB323J12201 NCT06581198 EUCT-2023-510150-17-00 JRCT2013240039 U1111-1309-0426 202500083 HumRes66631 SNCTP000006347 BASEC2024-02083	Phase II	In follow-up	Inflammatory and immune system	Lupus Nephritis, Systemic Lupus Erythematosus	T cells	Yes, ex vivo	Lentivirus	Autologous	2024	Hammersmith Hospital, London Royal Hallamshire Hospital, Sheffield
Novartis AG	Phase IIb Study of Rapcabtagene Autoleucel in IIM Participants	CYTB323L12201 JRCT2013240052 EUCT-2024-514137-38-00	Phase II	In planning/set-up	Inflammatory and immune system	Myositis	T cells	Yes, ex vivo	Lentivirus	Autologous	2024	Unconfirmed UK Sites
Novartis AG	Phase II Study Evaluating Rapcabtagene Autoleucel in Participants with Severe Active GPA or MPA	CYTB323I12201 NCT06868290 JRCT2013240076 HumRes67059 HumRes67230 SNCTP000006563 BASEC2024-02441	Phase II	Recruiting	Inflammatory and immune system	ANCA-Associated Vasculitis	T cells	Yes, ex vivo	Lentivirus	Autologous	2025	Novartis Investigative Site, Cambridge Cambridge University Hospital, Cambridge
Novartis AG	Study to Assess Safety, Efficacy, and Cellular Kinetics of YTB323 in Generalized Myasthenia Gravis	CYTB323O12101 JRCT2053240204 EUCT-2024-513589-20-00	Phase I/II	Recruiting	Neurological	Myasthaenia Gravis	T cells	Yes, ex vivo	Lentivirus	Autologous	2025	Novartis Investigative Site, Sheffield Royal Hallamshire Hospital, Sheffield
Novartis AG	A Registry-based Post-authorization Safety, Real World, Observational, Prospective Study of Voretigene Neparvovec in Subjects with RPE65 Mutation-associated Inherited Retinal Dystrophy (PERCEIVE)	CLTW888A12401	Phase IV	Recruiting	Ophthalmology	Hereditary retinal dystrophy	N/A	Yes, in vivo	AAV	N/A	2019	Unspecified, registry study
Oragenics Inc	Efficacy, Safety and Tolerability of AG013 in Oral Mucositis Compared to Placebo When Administered Three times per Day	US-1162 GDC30007079 GDCT0238094 NCT03234465 AG013-ODOM-201 NCI-2018-01381 EudraCT-2016-004161-68 B/BE/18/BVW3	Phase II	Terminated	Oral	Oral Mucositis (OM)	Other	Yes, ex vivo	N/A	N/A	2019	Queen Elizabeth Hospital, Birmingham Beatson West of Scotland Cancer Center, Glasgow Royal Cornwall Hospital, Truro Derriford Hospital, Plymouth The Christie NHS Foundation Trust, Manchester Guy's Hospital, Guy's and St Thomas' Foundation Trust, London
Orchard Therapeutics Ltd	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 EUCT-2022-500306-17-00 IND 28951	Phase III	In follow-up	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

Sponsor	Title	Clinical Database Numbers	Trial Phase	Trial Status	Disease Area	Indication	Cell Type	Gene Modification / Gene Therapy	Type of Vector Used (if applicable)	Autologous/ Allogeneic	Year Trial Started	United Kingdom Site(s)
Ormond Medical Arts Pharmaceutical Research Center	Lentiviral Gene Therapy for p47 AR-CGD	19IC05	Phase I/II	Recruiting	Genetic Disorders	Chronic Granulomatous Disease	CD34 and/or CD133 stem cells	Yes, <i>ex vivo</i>	Lentivirus	Autologous	2023	Great Ormond Street Hospital for Children NHS Foundation Trust, London
Passage Bio Inc	Study of Safety, Tolerability and Efficacy of PBKR03 in Pediatric Subjects with Early Infantile Krabbe Disease (GALax-C)	GDC30020961 PBKR03-001 EudraCT-2020-005229-95	Phase I/II	Suspended	Neurological	Krabbe Disease	N/A	Yes, <i>in vivo</i>	AAV	N/A	2022	Manchester University NHS Foundation Trust, Manchester
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)	NCT03861273 C0371002 EudraCT-2018-003086-33 JapicCTI-205228 jRCT1080225132 EUCT-2022-502844-11-00 202300481 KCT0008736 2023-03-019 2022-502844-11-00 IND-016437	Phase III	In follow-up	Haematological	Hemophilia B (Factor IX Deficiency)	N/A	Yes, <i>in vivo</i>	AAV	N/A	2019	Newcastle Upon Tyne Hospitals NHS Foundation Trust, Newcastle Upon Tyne Freeman Hospital, 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 EudraCT-2019-002921-31 MOH_2020-07-07_009124 KCT0005683 02-2020-027 2020-06-142 FP00018226 00127944 jRCT2051210011 SNCTP000004472 BASEC2020-02024 IRAS- 275551 2019-002921-31-GB 2023-508510-42-00 EUCT-2023-508510-42-00 CTIS2023-508510-42-00	Phase III	In follow-up	Musculoskeletal	Duchenne Muscular Dystrophy	N/A	Yes, <i>in vivo</i>	AAV	N/A	2020	Great Ormond Street Hospital for Children NHS Foundation Trust, London Liverpool University Hospitals NHS Foundation Trust, Liverpool Royal Victoria Infirmary, Alder Hey Children's NHS Foundation Trust, Liverpool
Pfizer Inc	Study to Evaluate the Efficacy and Safety of PF-07055480 / Giroctocogene Fitelparvovec Gene Therapy in Moderately Severe to Severe Hemophilia A Adults (AFFINE)	NCT04370054 C3731003 EudraCT-2019-004451-37 KCT0005828 202100117 EUCT-2024-512075-12-00 2024-512075-12-00 (CTIS) JPRN-jRCT2043210021	Phase III	In follow-up	Haematological	Haemophilia A	N/A	Yes, <i>in vivo</i>	AAV	N/A	2020	Guy's and St Thomas' NHS Foundation Trust, London
Pfizer Inc	A Study to Evaluate the Efficacy and Safety of Factor IX Gene Therapy with PF-06838435 in Pediatric Male Participants With Moderately Severe to Severe Hemophilia B (BeneGene-3)	2020-002443-38 C0371006	Phase III	Completed	Oncology (Haematology)	Hemophilia B (Factor IX Deficiency)	N/A	Yes, <i>in vivo</i>	AAV	N/A	2021	Glasgow Royal Infirmary, Glasgow Guy's and St Thomas' NHS Foundation Trust, London Newcastle Upon Tyne Hospitals NHS Foundation Trust, Newcastle Upon Tyne

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)
Pluri Inc	Efficacy, Tolerability and Safety of Intramuscular Injections of PLX PAD for the Treatment of Subjects with Critical Limb Ischemia (CLI) with Minor Tissue Loss Who Are Unsuitable for Revascularization	PLX-CLI-03 NCT03006770 EudraCT-2015-005532-18 GDCT0238084 GDC30007076 MOH_2018-07-08_002430	Phase III	Terminated	Cardiovascular	Peripheral Arterial Disease (PAD)/ Peripheral Vascular Disease (PVD), Critical Limb Ischemia	Mesenchymal stem/stromal cells	No	N/A	Allogeneic	2018	Hull University Teaching Hospitals NHS Trust, Hull St George's Hospital, London King's College Hospital, London Southmead Hospital, Bristol
Pluri Inc	Treatment of Muscle Injury Following Arthroplasty for Hip Fracture (HF)	NCT03451916 PLX-HF-01 GDC20008900 GDCT0267767 EudraCT-2017-005165-49 MOH_2018-05-31_002412	Phase III	Completed	Musculoskeletal	Muscle Injury	Mesenchymal stem/stromal cells	No	N/A	Allogeneic	2018	Norfolk and Norwich University Hospital, Norwich John Radcliffe Hospital, Oxford University of Warwick, Warwick University of Oxford, Oxford
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)	Gaucher Disease Type II	N/A	Yes, <i>in vivo</i>	AAV	N/A	2021	Manchester Centre for Genomic Medicine, Manchester
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 J4B-MC-OKAA (PRV-FTD101) EUCT-2022-502942-29-01	Phase I/II	Recruiting	Neurological	Frontotemporal Dementia	N/A	Yes, <i>in vivo</i>	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 IRAS ID-1005513 EUCT-2022-500281-10-02	Phase I/II	Recruiting	Metabolic (Other)	Gaucher Disease	N/A	Yes, <i>in vivo</i>	AAV	N/A	2022	Royal Free Hospital, London
Prime Medicine Inc	A Study of the Safety and Efficacy of Prime Editing (PM359) in Participants with P47phox Autosomal Recessive Chronic Granulomatous Disease (CGD)	GDC30035514 ; NCT06559176; Prime-0101	Phase I/II	Terminated	Genetic Disorders	Chronic Granulomatous Disease	CD34 and/or CD133 stem cells	Yes, <i>ex vivo</i>	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	Recruiting	Oncology (Other)	Head And Neck Cancer, Solid Tumor, Head And Neck Squamous Cell Carcinoma (HNSC)	N/A	Yes, <i>in vivo</i>	Adenovirus	N/A	2024	The Royal Marsden Hospital NHS Foundation Trust, London

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)
PulseSight Therapeutics SAS	Evaluation of EYS606 in Patients with Non-infectious Posterior, Intermediate or Panuveitis	EYS606-CT1 NCT03308045 208162 IRAS-208162 EudraCT-2015-001391-22	Phase I/II	Completed	Ophthalmology	Posterior Uveitis Intermediate Uveitis	N/A	Yes, <i>in vivo</i>	Non-viral	N/A	2017	University of Birmingham, Birmingham Bristol Eye Hospital, University Hospitals Bristol and Weston NHS Foundation Trust, Bristol Moorfields Eye Hospital NHS Foundation Trust, London
Purespring Therapeutics Ltd	A Phase I/II Clinical Study of PS-002 in Patients with Primary IgA Nephropathy	NCT07182227 PS-002-101 EUCT-2025-523201-14-00	Phase I/II	Recruiting	Renal and Urogenital	Primary IgA Nephropathy	N/A	Yes, <i>in vivo</i>	AAV	N/A	2025	Manchester University Hospital, Manchester University NHS Foundation Trust, Manchester Salford Royal NHS Foundation Trust, Salford Royal Infirmary of Edinburgh, Edinburgh Addenbrooke's Hospital, Cambridge University Hospitals NHS Foundation Trust, Cambridge Royal London Hospital, London Southmead Hospital, Bristol Leicester General Hospital, Leicester Nottingham University Hospitals NHS Trust, Nottingham Cardiff and Vale University Health Board, Cardiff
Queen Mary University of London	Use of Bone Marrow Derived Stem Cell and G-CSF with Circulatory Assistance in the Treatment of DCM (DCM-Support)	Reda 012357 EudraCT-2018-001063-23	Phase II	Recruiting	Cardiovascular	Dilated Cardiomyopathy	Bone marrow mononuclear cells	No	N/A	Autologous	2018	St Bartholomew's Hospital, London
Quell Therapeutics Ltd	Safety and Clinical Activity of QEL-001 in A2-mismatch Liver Transplant Patients (LIBERATE)	NCT05234190 QEL-001-CLN-01 EudraCT-2021-001379-18 EUCT-2024-516193-30-01 2024-516193-30-01	Phase I/II	Recruiting	Gastrointestinal (Liver)	Liver transplant rejection	T cells	Yes, <i>ex vivo</i>	Lentivirus	Autologous	2022	Cambridge University Hospitals NHS Foundation Trust, Cambridge Royal Free Hospital, London King's College Hospital NHS Foundation Trust, London Queen Elizabeth Hospital, Birmingham
Recode Therapeutics Inc	Study Evaluating the Safety and Tolerability of RCT1100 in Healthy and PCD Subjects	RCT1100-101 GDCT0480383	Phase I	Completed	Respiratory	Primary Ciliary Dyskinesia	N/A	Yes, <i>in vivo</i>	Non-viral	N/A	2023	Royal Brompton Hospital, London
Recode Therapeutics Inc	A Study to Assess the Safety, Tolerability, Ciliary Rescue, and Pharmacodynamics of RCT1100 in Adults with PCD	NCT06600425 RCT1100-102	Phase I	Completed	Respiratory	Primary Ciliary Dyskinesia	N/A	Yes, <i>in vivo</i>	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)	2024-511342-40-00 DB-OTO-001 EUCT-2024-511342-40-00 EudraCT-2022-000079-38 GDC30032310 GDCT0477424 IRAS-1005935 NCT05788536 2024-511342-40-00 (EU Trial (CTIS) Number)	Phase I/II	Recruiting	Ear, Nose, and Throat	Hearing Loss Due to Otoferlin Mutations (CHORD)	N/A	Yes, <i>in vivo</i>	AAV	N/A	2023	Addenbrooke's Hospital, Cambridge University Hospitals NHS Foundation Trust, Cambridge Great Ormond Street Hospital for Children NHS Foundation Trust, London

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)
Regeneron Pharmaceuticals Inc	A Study to Investigate the Safety and Effectiveness of a Coagulation Factor IX Gene Insertion Therapy (REGV131-LNP1265) in Pediatric, Adolescent and Adult Participants with Hemophilia B (BEYOND-9)	NCT06379789 R131L1265-HEMB-2318 2023-507260-40-00 IRAS-1009750	Phase I/II	Recruiting	Haematological	Hemophilia B (Factor IX Deficiency)	N/A	Yes, <i>in vivo</i>	AAV	N/A	2024	Addenbrooke's Hospital, Cambridge University Hospitals NHS Foundation Trust, Cambridge Hammersmith Hospital Comprehensive Care Centre, London St Thomas' Hospital, Guy's and St Thomas' NHS Foundation Trust, London Royal Free London NHS Foundation Trust, London Royal London Hospital, London Glasgow Royal Infirmary, Glasgow Queen Elizabeth Hospital Birmingham, Birmingham
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 - 303513 2024-511237-35-00 EUCT-2024-511237-35-00	Phase I/II	In follow-up	Neurological	Parkinson's Disease	Other	No	N/A	Allogeneic	2022	Cambridge University Hospitals NHS Foundation Trust, Cambridge
ReNeuron Group Plc	Pilot Investigation of Stem Cells in Stroke (PISCES)	RN01-CP-0001 UKCRN-9475	Phase I	Completed	Cardiovascular	Stable Ischemic Stroke	Neural	Yes, <i>ex vivo</i>	Retrovirus	Allogeneic	2010	Queen Elizabeth University Hospital, Glasgow
ReNeuron Group Plc	Safety and Tolerability of hRPC in Retinitis Pigmentosa (hRPCRP)	RN03-CP-0001 GDC40000507 GDCT0184314 EudraCT-2019-004547-77 2019-004547-77 IRAS-279052	Phase I/II	Completed	Ophthalmology	Retinitis Pigmentosa (Retinitis)	Retinal	No	N/A	Allogeneic	2015	Oxford Eye Hospital, Oxford
Replimune Ltd	Study of RP3 Monotherapy and RP3 in Combination with Nivolumab in Patients with Solid Tumours	RP3-301 GDC30019214 EUCT-2024-512710-17-00 2020-002870-29 NCT04336241	Phase I	Terminated	Oncology (Other)	Advanced Solid Tumor	N/A	Yes, <i>in vivo</i>	Oncolytic Virus	N/A	2020	The Clatterbridge Cancer Centre NHS Foundation Trust, Liverpool Churchill Hospital, Oxford University Hospitals NHS Foundation Trust, Oxford The Royal Marsden Hospital NHS Foundation Trust, Sutton
Replimune Ltd	Study of RP1 Monotherapy and RP1 in Combination with Nivolumab (IGNYTE)	EudraCT-2016-004548-12 NCT03767348 RPL-001-16 20180802 S17-01386 NCI-2019-01331	Phase I/II	Recruiting	Oncology (Other)	Advanced or Metastatic Cutaneous Squamous Cell Carcinoma, Angiosarcoma, Urothelial Cell Carcinoma, Metastatic Breast Cancer, Metastatic Uveal Melanoma, Ocular Melanoma, Acral Lentiginous Melanoma, Metastatic Bladder Cancer, Non-Small Cell Lung Cancer, Basal Cell Carcinoma, Squamous Cell Carcinoma, Merkel Cell Carcinoma, Cutaneous T-cell Lymphoma, Colorectal and Esophageal Cancers	N/A	Yes, <i>in vivo</i>	Oncolytic Virus	N/A	2017	The Clatterbridge Cancer Centre NHS Foundation Trust, Liverpool Southampton General Hospital, Southampton Oxford University Hospitals NHS Foundation Trust, Oxford Leeds Teaching Hospitals NHS Trust, Leeds The Royal Marsden Hospital 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 2024-512866-32-00 EUCT-2024-512866-32-00	Phase I	Recruiting	Oncology (Other)	Advanced Solid Tumor	N/A	Yes, <i>in vivo</i>	Oncolytic Virus	N/A	2019	The Clatterbridge Cancer Centre NHS Foundation Trust, Liverpool The Royal Marsden Hospital NHS Foundation Trust, London Churchill Hospital, Oxford University Hospitals NHS Foundation Trust, Oxford

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)
Resolution Therapeutics Ltd	RTX001 Autologous Engineered Macrophages for Liver Cirrhosis (Emerald)	EUCT-2024-516288-10-00 RTX001-002 NCT06823713 2024-516288-10 (EudraCT Number)	Phase I/II	Recruiting	Gastrointestinal (Liver)	Decompensated Cirrhosis Liver Diseases Decompensated Cirrhosis Liver Diseases	Other	No	Non-viral	Autologous	2024	Royal Infirmary of Edinburgh, Edinburgh
RHEACELL GmbH & Co KG	Allogeneic ABCB5-positive Stem Cells for Treatment of PAOD	NCT03339973 2017-000235-14 EudraCT-2017-000235-14	Phase I/II	Terminated	Cardiovascular	Wounds, Peripheral Arterial Disease (PAD)/ Peripheral Vascular Disease (PVD), Peripheral Artery Occlusive Disease (PAOD)	Mesenchymal stem/stromal cells	No	N/A	Allogeneic	2018	East Surrey Hospital, Surrey and Sussex Healthcare NHS Trust, Redhill
RHEACELL GmbH & Co KG	Allogeneic ABCB5-positive Stem Cells for Treatment of Epidermolysis Bullosa	allo-APZ2-EB-II-01 2018-001009-98 EudraCT-2018-001009-98	Phase I/II	Completed	Dermatological	Epidermolysis Bullosa (EB)	Mesenchymal stem/stromal cells	No	N/A	Allogeneic	2019	Great Ormond Street Hospital for Children NHS Foundation Trust, London King's College Hospital NHS Foundation Trust, London
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 EUCT-2024-512720-11-00	Phase III	In follow-up	Cardiovascular	Venous Leg Ulcers (Crural Ulcer)	Mesenchymal stem/stromal cells	No	N/A	Allogeneic	2024	RHEACELL Clinical Trial Sites, UK
Rinri Therapeutics Ltd	First in Human Randomised Trial of Rincell-1 in Adults with a Cochlear Implant	NCT07032038 R1-25 GDC30038385 GDCT0556741	Phase I/II	In planning/set-up	Ear, nose, and throat	Neural hearing loss, Postsynaptic auditory neuropathy , Presbycusis	Neural	No	N/A	Allogeneic	2025	Cambridge University Hospitals NHS Foundation Trust, Cambridge Guy's and St Thomas' Hospitals NHS Foundation Trust, London University Hospitals Birmingham NHS Foundation Trust, Birmingham
Rocket Pharmaceuticals Inc	Lentiviral-mediated Gene Therapy for Pediatric Patients With Fanconi Anemia Subtype A	EudraCT-2018-002502-31 ; RP-L102-0118; NCT04069533; GDC30018249; GDCT033058	Phase I/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	A Clinical Trial to Evaluate the Safety and Efficacy of RP-L201 in Subjects with Leukocyte Adhesion Deficiency-I	NCT03812263 RP-L201-0318 GDC30020285 GDCT0349494 2020-000517-33 EudraCT-2020-000517-33 XX-0054	Phase I/II	Completed	Inflammatory and immune system	Leukocyte Adhesion Deficiency-I (LAD-I)	CD34 and/or CD133 stem cells	Yes, ex vivo	Lentivirus	Autologous	2019	Great Ormond Street Hospital for Children NHS Foundation Trust, London UCL Great Ormond Street Institute of Child Health, London

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	Lentiviral-mediated Gene Therapy for Pediatric Patients With Fanconi Anemia Subtype A	RP-L102-0118 2018-002502-31 NCT04069533 GDC30018249 GDCT0330589	Phase II	Completed	Haematological	Fanconi Anemia	CD34 and/or CD133 stem cells	Yes, <i>ex vivo</i>	Lentivirus	Autologous	2019	Great Ormond Street Hospital for Children NHS Foundation Trust, London
Rocket Pharmaceuticals Inc	A Gene Therapy Study of RP-A501 in Male Patients with Danon Disease	NCT06092034 RP-A501-0123 GDCT0501290 2023-506480-34-00 EUCT-2023-506480-34-00	Phase II	Recruiting	Metabolic (Other)	Danon Disease	N/A	Yes, <i>in vivo</i>	AAV	N/A	2023	Great Ormond Street Hospital for Children NHS Foundation Trust, London
Rocket Pharmaceuticals Inc	Long-term Follow-up: Phase I/II Clinical Study to Evaluate the Safety and Efficacy of the Infusion of RP-L102	RP-L102-0221-LTFU	Phase I/II	Recruiting	Haematological	Fanconi Anaemia	CD34 and/or CD133 stem cells	No	N/A	Autologous	2023	University College London Hospitals NHS Foundation Trust, London 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 (LAD-I)	CD34 and/or CD133 stem cells	Yes, <i>ex vivo</i>	Lentivirus	Autologous	2022	Great Ormond Street Hospital for Children NHS Foundation Trust, London
Sangamo Therapeutics Inc	Long-Term Follow-Up of TX200-TR101 (STEADFAST)	EudraCT-2022-002440-40 TX200-KT03 NCT05987527 EUCT-2024-512580-31-00	Phase III	Recruiting	Renal and Urogenital	Kidney Transplant Rejection	T cells	Yes, <i>ex vivo</i>	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 2019-000667-24 EudraCT-2019-000667-24 s18-02063 IRAS-261195 EUCT-2024-512695-34-00 U1111-1305-1998	Phase I/II	In follow-up	Metabolic (Other)	Fabry Disease	N/A	Yes, <i>in vivo</i>	AAV	N/A	2019	Queen Elizabeth Hospital, Birmingham Addenbrooke's Hospital, Cambridge Royal Free Hospital, London
Sangamo Therapeutics Inc	Ascending Dose Study of Genome Editing by Zinc Finger Nuclease Therapeutic SB-FIX in Subjects with Severe Hemophilia B	SB-FIX-1501 NCT02695160 15420 2017-004805-42	Phase I/II	Terminated	Haematological	Hemophilia B (Factor IX Deficiency) Mucopolysaccharidosis I (MPS I) (Hurler Syndrome)	N/A	Yes, <i>in vivo</i>	AAV	N/A	2016	Hammersmith Hospital, London Queen Elizabeth Hospital, Birmingham Glasgow Royal Infirmary, Glasgow

Sponsor	Title	Clinical Database Numbers	Trial Phase	Trial Status	Disease Area	Indication	Cell Type	Gene Modification / Gene Therapy	Type of Vector Used (if applicable)	Autologous/ Allogeneic	Year Trial Started	United Kingdom Site(s)
Sangamo Therapeutics Inc	Safety and Tolerability Study of Chimeric Antigen Receptor T-Reg Cell Therapy in Living Donor Renal Transplant Recipients (STeakfast)	EUCT-2024-512579-11-00 EudraCT-2019-001730-34 NCT04817774 TX200 KT02 U1111-1305-2706	Phase I/II	In follow-up	Renal and Urogenital	Kidney Transplant Rejection	T cells	Yes, <i>ex vivo</i>	Lentivirus	Autologous	2022	Oxford University Hospitals NHS Foundation Trust, Oxford
Sangamo Therapeutics Inc	Long-term Follow-up of Subjects who were Treated with ST-920	ST-920-LT01 IRAS-1005663 2022-001411-86 EudraCT-2022-001411-86 2024-512700-18-00 EUCT-2024-512700-18-00	Phase I/II	Recruiting	Metabolic (Other)	Fabry Disease	N/A	Yes, <i>in vivo</i>	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) (EMBARC)	Study 301 SRP-9001-301 NCT05096221 JRCT2053210141 EudraCT-2019-003374-91 P/419/2020 9001-301	Phase III	Completed	Musculoskeletal	Duchenne Muscular Dystrophy	N/A	Yes, <i>in vivo</i>	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 Gene Transfer Therapy Study to Evaluate the Safety and Efficacy of Delandistrogene Moxeparvovec (SRP-9001) in Non-Ambulatory and Ambulatory Participants With Duchenne Muscular Dystrophy (DMD) (ENVISION)	Study 303 EudraCT-2020-002372-13 SRP-9001-303 P/052/2022 NCT05881408 JRCT2033230274 EUCT-2024-512626-28-00 2024-512626-28-00 EMA-094156-PIP52-22	Phase III	In follow-up	Musculoskeletal	Duchenne Muscular Dystrophy	N/A	Yes, <i>in vivo</i>	AAV	N/A	2023	Great Ormond Street Hospital for Children NHS Foundation Trust, London Translational and Clinical Research Institute, Newcastle University, Newcastle Upon Tyne Churchill Hospital, Oxford University Hospitals NHS Foundation Trust, Oxford
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 Study 9001-305 Study 305 JRCT2053240056 EUCT-2023-505043-39-00 U1111-1296-0489 2023-505043-39-00 (CTIS) OCR45013 23-000873 JPRN-JRCT2053240056	Phase III	Recruiting	Genetic Disorders	Duchenne Muscular Dystrophy	N/A	Yes, <i>in vivo</i>	AAV	N/A	2023	Great Ormond Street Hospital For Children NHS Foundation Trust, London Translational and Clinical Research Institute, Newcastle University, Newcastle Upon Tyne Oxford University Hospitals NHS Foundation Trust, Oxford
Sarepta Therapeutics Inc	A Trial to Learn More About an Experimental Gene Therapy Called Bidridistrogene Xeboparvovec (SRP-9003) as a Possible Treatment for Limb Girdle Muscular Dystrophy 2E/R4 (EMERGENE)	GDC30032534 SRP-9003-301 EudraCT-2022-503112-17-00 1009294	Phase III	Suspended	Genetic Disorders	Limb-Girdle Muscular Dystrophy type 2E (LGMD2E)	N/A	Yes, <i>in vivo</i>	AAV	N/A	2024	Newcastle University, Newcastle Upon Tyne

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	SCIB1 and iSCIB1+ in Melanoma Patients Receiving Nivolumab with Ipilimumab or SCIB1 with Pembrolizumab (The SCOPE Study)	GDCT0366648 SCIB1-002 EUCT-2018-002844-10 IND 18331 CRUK 16281 GDC30009247	Phase II	Recruiting	Oncology (Other)	Advanced Unresectable Melanoma	N/A	Yes, <i>in vivo</i>	Non-viral	N/A	2019	Cambridge University Hospitals NHS Foundation Trust, Cambridge Addenbrooke's Hospital, Cambridge Southampton Clinical Trials Unit, Maybush The Royal Marsden Hospital NHS Foundation Trust, London The Christie NHS Foundation Trust, Manchester Velindre University NHS Trust, Cardiff Weston Park Hospital, Sheffield Musgrove Park Hospital, Taunton Derriford Hospital, Plymouth Royal Derby Hospital, Derby St James' University Hospital, Leeds University Hospital Plymouth NHS Trust, Plymouth Royal Free London NHS Foundation Trust, London Guy's & St Thomas' NHS Foundation Trust, London University Hospital Southampton NHS Foundation Trust, Southampton Mount Vernon Hospital, Northwood Nottingham University Hospitals NHS Trust, Nottingham Lancashire Teaching Hospitals NHS Foundation Trust, Preston East and North Hertfordshire NHS Trust, Northwood, Middlesex Churchill Hospital, Oxford University Hospitals NHS Foundation Trust, Oxford Oxford University Hospital NHS Foundation Trust, Oxford Somerset NHS Foundation Trust, Taunton Sheffield Teaching Hospital NHS Foundation Trust, Sheffield Leeds Teaching Hospitals NHS Trust, Leeds
Scarcell Therapeutics SAS	FIH Safety and Feasibility Study Assessing Intra-articular Administration of aeGF in Patients with Knee Osteoarthritis	GF-OA-001	Phase I	Recruiting	Musculoskeletal	Osteoarthritis	Fibroblasts	No	N/A	Allogeneic	2025	Oxford University Hospitals NHS Foundation Trust, Oxford
Servier Institute of International Research	Dose Escalation Study of UCART19 in Adult Patients with Relapsed / Refractory B-cell Acute Lymphoblastic Leukaemia (CALM)	NCT02746952 CL1-68587-002 2016-000296-24 EudraCT-2016-000296-24 NCI-2017-01223 CDR789815 IRAS 200594 2016-0725 UKCTG-21148 JapicCTI-195059 JRCT2013190002 JRCT1080224971	Phase I	Completed	Oncology (Haematology)	B-cell acute lymphoblastic leukaemia (B-ALL)	T cells	Yes, <i>ex vivo</i>	Lentivirus	Allogeneic	2016	The Christie NHS Foundation Trust, Manchester King's College Hospital NHS Foundation Trust, London
Servier Institute of International Research	Study of UCART19 in Pediatric Patients with Relapsed/refractory B Acute Lymphoblastic Leukemia (PALL)	NCT02808442 2015-004293-15 IRAS-200331 UCART19_02 (CL1-68587-001) UKCTG-25377 ES-0049 US-1786	Phase I	Completed	Oncology (Haematology)	Relapsed/Refractory B Acute Lymphoblastic Leukemia	T cells	Yes, <i>ex vivo</i>	Lentivirus	Allogeneic	2016	Great Ormond Hospital for Children NHS Foundation Trust, London
Sillajen Biotherapeutics	Hepatocellular Carcinoma Study Comparing Vaccinia Virus Based Immunotherapy Plus Sorafenib vs Sorafenib Alone (PHOCUS)	NCT02562755 JX594-HEP024 NCI-2016-00198 CDR779028 EudraCT-2014-001985-86 200385 IRAS-200385 UCI-15-80 STU 102015-040 OSU-15255 CTR20171652 GDCT0256326 GDC20007048 MOH_2017-03-08_000218 1504-1425 XX-0037	Phase III	Completed	Oncology (Other)	Hepatocellular Carcinoma (HCC)	N/A	Yes, <i>in vivo</i>	Oncolytic Virus	N/A	2015	Site No. 9501, Birmingham Site No. 9505, Guildford Site No. 9503, Leeds Site No. 9502, London Site No. 9504, London Site No. 9506, London

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)
Sio Gene Therapies Inc	Long Term Safety and Efficacy Study of ProSavin in Parkinson's Disease	PS1/001/09 EudraCT-2009-017253-35 2009-017253-35	Phase I/II	Terminated	Neurological	Parkinson's Disease	N/A	Yes, <i>in vivo</i>	Lentivirus	N/A	2011	Addenbrooke's Hospital, Cambridge
Sio Gene Therapies Inc	Study of OXB-102 (AXO-Lenti-PD) in Patients with Bilateral, Idiopathic Parkinson's Disease (SUNRISE-PD)	NCT03720418 OXB-102-01 FOX-006139 006139	Phase I/II	Terminated	Neurological	Parkinson's Disease	N/A	Yes, <i>in vivo</i>	Lentivirus	N/A	2018	Centre for Brain Repair, University of Cambridge, Cambridge The National Hospital for Neurology and Neurosurgery, London University College London Hospitals NHS Foundation Trust, London National Institute for Health Research, Southampton
Solid Biosciences Inc	A Study of SGT-003 Gene Therapy in Duchenne Muscular Dystrophy (INSPIRE DUCHENNE)	NCT06138639 SGT-003-101 EUCT-2024-514501-57-00 29667 285259 1010251 GDCT0503991	Phase I/II	Recruiting	Musculoskeletal	Duchenne Muscular Dystrophy	N/A	Yes, <i>ex vivo</i>	AAV	N/A	2024	Great Ormond Street Hospital for Children NHS Foundation Trust, London
SOTIO Biotech AS	Phase III Study of DCVAC/PCa Added to Standard Chemotherapy for Men With Metastatic Castration Resistant Prostate Cancer (VIABLE)	SP005 NCT02111577 CDR759943 DRKS00006380 NCI-2014-02085 IRAS108971 AUO-Studie AP 78/13 20140714 2012-002814-38	Phase III	Completed	Oncology (Other)	Prostate Cancer, Metastatic Castration-Resistant Prostate Cancer (mCRPC), Metastatic Prostate Cancer	Dendritic cells	No	N/A	Autologous	2014	The Clatterbridge Cancer Centre, Liverpool University Hospitals Birmingham NHS Foundation Trust, Birmingham Bristol Haematology and Oncology Centre, University Hospitals Bristol and Weston NHS Foundation Trust, Bristol Cambridge University Hospitals NHS Foundation Trust, Cambridge University of Surrey, Guildford Royal Free Hospital, London The Royal Marsden Hospital NHS Foundation Trust, London The Christie NHS Foundation Trust, Manchester Freeman Hospital, Newcastle Upon Tyne
SOTIO Biotech AS	BOXR1030 T Cells in Subjects With Advanced GPC3-Positive Solid Tumors (DUET-1)	B1030-101 ISRCTN15110275 1005273 IRAS1005273 CPMS 52312 EudraCT-2021-005086-41 IND-27903 NCI-2022-02422	Phase I/II	In follow-up	Oncology (Other)	Solid tumours	T cells	Yes, <i>ex vivo</i>	Lentivirus	Autologous	2022	The Royal Marsden Hospital NHS Foundation Trust, London University College London Hospitals NHS Foundation Trust, London Cardiff and Vale University Health Board, Cardiff Beatson Institute for Cancer Research, Glasgow
Spark Therapeutics Inc	A Gene Transfer Study for Late-Onset Pompe Disease (RESOLUTE)	GDC30018726 SPK-3006-101 EUCT-2019-001283-30 EUCT-2023-509303-32-00	Phase I/II	Recruiting	Metabolic (Other)	Lysosomal Storage Disorder	N/A	Yes, <i>in vivo</i>	AAV	N/A	2020	Salford Royal NHS Foundation Trust, Salford Royal Free London NHS Foundation Trust, London Queen Elizabeth Hospital, Birmingham
Spur Therapeutics Ltd	A Long-Term Follow-Up Study of Haemophilia B Patients Who Have Undergone Gene Therapy	FLT180a-04 2017-005080-40 EudraCT-2017-005080-40 IRAS 241903	Phase I/II	Terminated	Haematological	Haemophilia B	N/A	Yes, <i>in vivo</i>	AAV	N/A	2018	Royal Free London NHS Foundation Trust, London Southampton Haemophilia Comprehensive Care Centre, Southampton Newcastle Haemophilia Comprehensive Care Centre, Newcastle Oxford University Hospitals NHS Foundation Trust, Oxford Kent and Canterbury Hospital, Canterbury

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)
Spur Therapeutics Ltd	A Fabry Disease Gene Therapy Study (Marvel1)	NCT04040049 FLT190-01 EudraCT-2018-002097-51 XX-0077	Phase I/II	Terminated	Genetic Disorders	Fabry Disease	N/A	Yes, <i>in vivo</i>	AAV	N/A	2019	Royal Free Hospital, London Salford Royal NHS Foundation Trust, Salford
Spur Therapeutics Ltd	A Long Term Follow-up Study of Fabry Disease Subjects Treated with FLT190	FLT190-02 2019-004645-32 EudraCT-2019-004645-32	Phase I/II	Completed	Metabolic (Other)	Fabry Disease Lysosomal Storage Disorders	N/A	Yes, <i>in vivo</i>	AAV	N/A	2020	Royal Free London NHS Foundation Trust, London
Spur Therapeutics Ltd	Phase I/II Dose Confirmation Study of FLT180a in Hemophilia B	NCT05164471 FLT180a-06 2021-001079-18 EudraCT-2021-001079-18	Phase I/II	Terminated	Genetic Disorders	Hemophilia B	N/A	Yes, <i>in vivo</i>	AAV	N/A	2021	Glasgow Royal Infirmary, Glasgow Guy's Hospital, Guy's and St Thomas' NHS Foundation Trust, London Royal Free London NHS Foundation Trust, London Royal Victoria Infirmary, Newcastle Upon Tyne
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	Completed	Metabolic (Other)	Gaucher Disease Type 1	N/A	Yes, <i>in vivo</i>	AAV	N/A	2022	Royal Free Hospital, London Salford Royal Hospital, Salford Queen Elizabeth Hospital, Birmingham
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	In follow-up	Metabolic (Other)	Gaucher Disease Type I	N/A	Yes, <i>in vivo</i>	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	NCT00979238 AGT4HB 5R01HL094396 EudraCT-2005-005711-17	Phase I/II	In follow-up	Haematological	Hemophilia B (Factor IX Deficiency)	N/A	Yes, <i>in vivo</i>	AAV	N/A	2010	Katharine Dormandy Haemophilia Centre and Haemostasis Unit, University College of London, London St George's Hospital, London University College London Hospitals NHS Foundation Trust, London University of Southampton, Southampton Bupa foundation, London
Swansea Bay University Health Board	A Single-centered, Prospective, Observational Cohort Study on Longitudinal Changes in Quality of Life after CAR-T Therapy in Subjects with Non-hodgkin Lymphoma		Phase IV	Completed	Oncology (Haematology)	Non-Hodgkin Lymphoma	T cells	Yes, <i>ex vivo</i>	Retrovirus	Autologous	2019	University Hospital of Wales, Cardiff and Vale University Health Board, Cardiff

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 249620 IRAS 249620	Phase IV	Terminated	Gastrointestinal (Other)	Crohn's Disease and Complex Perianal Fistulas	Mesenchymal stem/stromal cells	No	N/A	Allogeneic	2018	St Mark's The National Bowel Hospital, London Northern General Hospital, Sheffield Teaching Hospitals NHS Foundation Trust, 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	In follow-up	Neurological	Rett Syndrome	N/A	Yes, <i>in vivo</i>	AAV	N/A	2023	Evelina London Children's Hospital, London
TC BioPharm Ltd	A Phase II Safety and Tolerability Study of TCB008 in Patients With COVID-19	TCB 008-002 GDCT0417564 GDC30024490	Phase II	Withdrawn	Respiratory	Coronavirus Disease (COVID-19)	T cells	No	N/A	Allogeneic	2021	Royal Victoria Infirmary, Newcastle Upon Tyne
TC BioPharm Ltd	ACHIEVE - Efficacy and Effectiveness of Adoptive Cellular Therapy with <i>Ex-Vivo</i> Expanded Allogeneic γδ T-lymphocytes (TCB008) for Patients With Refractory or Relapsed Acute Myeloid Leukaemia (AML) (ACHIEVE)	TCB008-001 NCT05358808	Phase II/III	Terminated	Oncology (Haematology)	Refractory/Relapsed Acute Myeloid Leukaemia	T cells	No	N/A	Allogeneic	2022	Bristol and Weston NHS Foundation Trust, Bristol Cardiff and Vale University Health Board, 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
Tern Therapeutics LLC	A First-in-Human, Open-Label, Dose-Escalation Study to Evaluate the Safety and Tolerability of Gene Therapy with TTX-381 for the Ocular Manifestations Associated with Neuronal Ceroid Lipofuscinosis Type 2 (CLN2) Disease	2021-000173-92 NCT05791864 TTX-381-1102 EUCT-2025-521175-31-00	Phase I/II	In follow-up	Genetic Disorders	Batten Disease, Unspecified Ophthalmological Disorders	N/A	Yes, <i>in vivo</i>	AAV	N/A	2023	Greater Ormond Street Hospital for Children NHS Foundation Trust, London
TETEC Tissue Engineering Technologies AG	Phase III Study to Evaluate Safety and Effectiveness of NOVOCART 3D Plus vs. Microfracture in Knee Cartilage Defects (N3D)	AAG-G-H-1202 EudraCT-2011-005798-22 IRAS-151121	Phase III	Completed	Musculoskeletal	Articular Cartilage Defect	Chondrocytes	No	N/A	Autologous	2013	University Hospital of Coventry and Warwickshire, Coventry Heart of England NHS Foundation Trust, Birmingham Spire little Aston Hospital, Birmingham University of Warwick, Coventry Nuffield Health Bournemouth Hospital, Bournemouth ORION Clinical Services Ltd, Worcestershire Droitwich Knee Clinic, Worcestershire
The Robert Jones & Agnes Hunt Orthopaedic Hospital NHS Foundation Trust	Autologous Stem Cells, Chondrocytes Or the Two (ASCOT)	R5242 A789 EudraCT-2010-022072-31 IRAS 50345 18480 19429 21156 MR/L010453/1 R5242A789 50345	Phase II	In follow-up	Musculoskeletal	Osteoarthritis	Chondrocytes	No	N/A	Autologous	2011	The Robert Jones and Agnes Hunt Orthopaedic Hospital NHS Foundation Trust, Oswestry

Sponsor	Title	Clinical Database Numbers	Trial Phase	Trial Status	Disease Area	Indication	Cell Type	Gene Modification / Gene Therapy	Type of Vector Used (if applicable)	Autologous/ Allogeneic	Year Trial Started	United Kingdom Site(s)
The Royal Marsden NHS Foundation Trust	TITAN (Tumoural Injection of T-VEC and Isolated Limb Perfusion) (TITAN)	CCR 4679	Phase I/II	Completed	Oncology (Other)	Melanoma	N/A	Yes, <i>in vivo</i>	Oncolytic Virus	N/A	2018	The Royal Marsden Hospital NHS Foundation Trust, London
The University of Manchester	Mesoangioblast-Based Gene Therapy For Duchenne Muscular Dystrophy:A Phase I/IIa Study / DMD04	R119513 WT200134/Z15/Z 2019-001825-28	Phase I/II	Recruiting	Musculoskeletal	Duchenne Muscular Dystrophy	Other	Yes, <i>ex vivo</i>	Lentivirus	Autologous	2019	Royal Manchester Children's Hospital, Manchester
The University of Manchester	Gene Therapy with Modified Autologous Hematopoietic Stem Cells for Patients with Mucopolysaccharidosis Type II	ISRCTN12458940 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, <i>ex vivo</i>	Lentivirus	Autologous	2023	Central Manchester University Hospitals NHS Foundation Trust, Manchester Manchester Royal Infirmary, 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 EudraCT-2019-002051-42 IRAS ID 265529	Phase I/II	Completed	Metabolic (Other)	Mucopolysaccharidosis IIIA (MPS IIIA) (Sanfilippo Syndrome) - Lysosomal storage disease	CD34 and/or CD133 stem cells	Yes, <i>ex vivo</i>	Lentivirus	Autologous	2020	Manchester University Hospital 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, London University Hospitals Coventry and Warwickshire NHS Trust, Coventry
Theolytics Ltd	Trial of THEO-260 in Ovarian Cancer Patients (OCTOPOD)	NCT06618235 THEO-260-001 2024-511236-27-00 EUCT-2024-511236-27-00 IRAS 1007880	Phase I/II	Recruiting	Oncology (Other)	Ovarian Cancer, Fallopian Tube Cancer, Endometrial Cancer, Peritoneal Cancer	N/A	Yes, <i>in vivo</i>	Oncolytic Virus	N/A	2024	Imperial College Healthcare NHS Trust, London Churchill Hospital, Oxford University Hospitals NHS Foundation Trust, Oxford The Beatson West of Scotland Cancer Centre, Glasgow
TiGenix NV	Study to Assess Efficacy and Safety of Cx601, Adult Allogeneic Expanded Adipose-derived Stem Cells (eASC) for the Treatment of Complex Perianal Fistula(s) in Participants with Crohn's Disease (CD) (ADMIRE-CD-II)	EudraCT-2017-000725-12 Cx601-0303 NCT03279081 Cx601-303 MOH_2018-05-09_001964 237254 21358	Phase III	Completed	Gastrointestinal (Other)	Crohn's Disease	Adipose-derived stem cells	No	N/A	Allogeneic	2017	Glasgow Royal Infirmary, Glasgow Addenbrooke's Hospital, Cambridge Wythenshawe Hospital, Manchester St Mark's Hospital, London Guy's and St Thomas' NHS Foundation Trust, London University College London Hospitals NHS Foundation Trust, London Nottingham University Hospitals NHS Trust, Nottingham Northern General Hospital, Sheffield
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	Phase I/II	Terminated	Oncology (Other)	Solid Tumor	T cells	Yes, <i>ex vivo</i>	Retrovirus	Autologous	2022	The Christie NHS Foundation Trust, Manchester

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	GDC30018610 TG4050.02 EudraCT-2018-003267-58	Phase I	Recruiting	Oncology (Other)	Squamous cell carcinoma of the head and neck	N/A	Yes, <i>in vivo</i>	MVA	N/A	2019	NHS Clatterbridge Cancer Center, Liverpool Aintree University Hospital NHS Foundation Trust, Liverpool University Hospital NHS Foundation Trust, Southampton
Transgene SA	Study of Intrahepatic Arterial Infusion of TG6002 in Combination with 5-FC in Patients with Metastatic Colorectal Cancer	2018-004103-39 EudraCT-2018-004103-39 TG6002.03 NCT04194034	Phase I/II	Terminated	Oncology (Other)	Colorectal Neoplasms	T cells	Yes, <i>in vivo</i>	Oncolytic Virus	N/A	2020	St James's University Hospital, Leeds
Ultragenyx Pharmaceutical Inc	Safety and Dose-finding Study of DTX301 (scAAV8OTC) in Adults with Late-onset Ornithine Transcarbamylase (OTC) Deficiency (CAPtivate)	NCT02991144 301OTC01 2016-001057-40 UKCTG-38256 IRAS-211800 1604-1520 17190 XX-0032	Phase I/II	Completed	Metabolic (Other)	Ornithine-Transcarbamylase Deficiency	N/A	Yes, <i>in vivo</i>	AAV	N/A	2017	Queen Elizabeth Hospital, Birmingham National Hospital for Neurology and Neurosurgery, London
Ultragenyx Pharmaceutical Inc	Long-term Safety, Tolerability, and Efficacy of DTX101 (AAVRH10FIX) in Adults with Moderate/Severe to Severe Hemophilia B	101HEMB02 EudraCT-2016-003430-25 2016-003430-25 IRAS 224520 IND-16543	Phase I/II	Completed	Haematological	Hemophilia B (Factor IX Deficiency)	N/A	Yes, <i>in vivo</i>	AAV	N/A	2017	Haemostasis & Thrombosis Centre, Basingstoke Manchester Haemophilia Comprehensive Care Center, Manchester
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 EUCT - 2022-501146-30-00 2018-000156-18 (EudraCT Number)	Phase I/II	In follow-up	Metabolic (Other)	Ornithine-Transcarbamylase Deficiency	N/A	Yes, <i>in vivo</i>	AAV	N/A	2018	Queen Elizabeth Hospital, Birmingham
Ultragenyx Pharmaceutical Inc	A Phase I/II/III Study of UX701 Gene Therapy in Adults with 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	Phase II/III	In follow-up	Genetic Disorders	Wilson Disease	N/A	Yes, <i>in vivo</i>	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 EUCT-2024-511766-37-00	Phase I/II	In follow-up	Neurological	Huntington Disease	N/A	Yes, <i>in vivo</i>	AAV	N/A	2021	University Hospital of Wales, Cardiff National Hospital for Neurology and Neurosurgery, London

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	CAR19 Donor Lymphocytes for Relapsed CD19+ Malignancies Following Allogeneic Transplantation (CARD)	UCL16/0045 2016-001869-85 EudraCT-2016-001869-85 IRAS-200484	Phase I	Completed	Oncology (Other)	CD19+ Malignancies	T cells	Yes, ex vivo	Lentivirus	Allogeneic	2017	University College London Hospitals NHS Foundation Trust, London
University College London	Gene Therapy for Haemophilia A (GO-8)	MR/L013185/1 EudraCT-2016-000925-38 UCL 13/0076 IRAS-207751	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	Infusion of Depleted T Cells Following Unrelated Donor Stem Cell Transplant (ICAT)	UCL/11/0519 MR/K007491/1 EudraCT-2013-000872-14 CDR749050 13/SC/0322 IRAS-131126	Phase I/II	Completed	Oncology (Haematology)	Acute Myeloid Leukemia/Acute Lymphocytic Leukemia	T cells	No	N/A	Allogeneic	2014	Manchester Royal Infirmary, Manchester University College London Hospitals NHS Foundation Trust, London
University College London	Evaluation of CAR19 T-cells as an Optimal Bridge to Allogeneic Transplantation (COBALT)	UCL/14/0385 Eudra CT-2015-000348-40 IRAS ID-173780 CRUK 12681	Phase I	Completed	Oncology (Other)	Diffuse Large B-Cell Lymphoma	T cells	Yes, ex vivo	Lentivirus	Autologous	2016	University College London Hospitals NHS Foundation Trust, London
University College London	Carpall: Immunotherapy with CD19+CD22 Car T-cells for CD19+ and CD22+ Acute Lymphoblastic Leukaemia	UCL 14/0529 EudraCT-2015-001144-10 IRAS 179225 GDC30021653 GDCT0366450 14118 CRUK-14118	Phase I	Recruiting	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
University College London	Immunotherapy with Tacrolimus Resistant EBV CTL for Lymphoproliferative Disease after Solid Organ Transplant (ITREC)	UCL/16/0529 2017-001020-22 IRAS-225911	Phase I	In follow-up	Oncology (Haematology)	Epstein-Barr Virus-Associated Post-Transplant Lymphoproliferative Disease	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	Targeted Stem Cells Expressing Trail as a Therapy for Lung Cancer	UCL/14/0453 228124 EudraCT-2015-005526-18	Phase I/II	Completed	Oncology (Other)	Lung Cancer, Non-Small Cell Lung Cancer, Lung Adenocarcinoma	Mesenchymal stem/stromal cells	Yes, ex vivo	Lentivirus	Allogeneic	2019	University College London Hospitals NHS Foundation Trust, London

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	A Factor IX Gene Therapy Study (FIX-GT) (FIX-GT)	UCL/15/0552 15/0552 2017-000852-24 EudraCT-2017-000852-24 UK-0234	Phase I/II	Terminated	Haematological	Hemophilia B (Factor IX Deficiency)	N/A	Yes, <i>in vivo</i>	AAV	N/A	2017	Basingstoke Haemostasis and Thrombosis Centre, Basingstoke Guy's and St Thomas' NHS Foundation Trust, London Newcastle Hospitals NHS Trust, Newcastle Royal Free Hospital, London Oxford University Hospital, Oxford University Hospital Southampton, Southampton East Kent Hospitals University, Canterbury Oxford University Hospital, Oxford University of Sheffield, Sheffield Manchester Royal Infirmary, Manchester
University College London	Multi-modular Chimeric Antigen Receptor Targeting GD2 in Neuroblastoma (MAGNETO)	NCT05990751 UCL\142424	Phase I	Recruiting	Oncology (Other)	Neuroblastoma	T cells	Yes, <i>ex vivo</i>	Retrovirus	Autologous	2024	Great Ormond Street Hospital for Children NHS Foundation Trust, London
University College London	Transfer of Effector Memory T Cells (Tem) Following Allogeneic Stem Cell Transplantation (ToTem)	UCL/13/0372 MR/R025436/1	Phase I	Recruiting	Oncology (Haematology)	Lymphoma, Leukemia, Myelodysplastic Syndrome, Aplastic Anemia, Primary Immune Deficiency (PID), Graft Versus Host Disease (GVHD), Non-Hodgkin Lymphoma Hodgkin Lymphoma (B-Cell Hodgkin Lymphoma), Chronic Lymphocytic Leukemia (CLL), Acute Myelocytic Leukemia (AML, Acute Myeloblastic Leukemia), Acute Lymphocytic Leukemia (ALL, Acute Lymphoblastic Leukemia), Multiple Myeloma (Kahler Disease), Chronic Myelomonocytic Leukemia (CMML)	T cells	No	N/A	Allogeneic	2019	University College London Hospitals 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	Recruiting	Oncology (Haematology)	Multiple Myeloma	T cells	Yes, <i>ex vivo</i>	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 (Haematology)	Primary CNS Lymphoma	T cells	Yes, <i>ex vivo</i>	Lentivirus	Autologous	2021	University College London Hospitals NHS Foundation Trust, London
University College London	Halting Ornithine Transcarbamylase Deficiency With Recombinant AAV in Children (HORACE)	NCT05092685 18/0123 ISRCTN47106904 2020-005342-41 IRAS 1003541 EudraCT-2020-005342-41	Phase I/II	Suspended	Genetic Disorders	Ornithine-Transcarbamylase Deficiency	N/A	Yes, <i>in vivo</i>	AAV	N/A	2023	Great Ormond Street Hospital for Children NHS Foundation Trust, London
University College London	CAR T Cells to Target GD2 for DMG (CARMIGO)	UCL/ 150853	Phase I	Recruiting	Oncology (Other)	Glioma	T cells	Yes, <i>ex vivo</i>	Retrovirus	Autologous	2023	Great Ormond Street Hospital for Children NHS Foundation Trust, London

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	Determining the Mechanisms of Loss of CAR T Cell Persistence (CARPERS)	UCL/156503	Phase IV	Recruiting	Oncology (Haematology)	Acute Lymphoblastic Leukaemia (ALL)	T cells	Yes, ex vivo	Lentivirus	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
University College London	A CAR T Study For Paediatric-Type Diffuse High-Grade Gliomas Including Diffuse Midline Glioma	1008429 UCL/150463	IRAS 1008429	Phase I	Recruiting	Oncology (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	Immunotherapy using CAR T-cells to target EGFRvIII for relapsed/refractory adult Glioblastoma	1007305 UCL/126897 CPMS 57214 EudraCT-2022-003747-10 IRAS 1007305	Phase I	Recruiting	Oncology (Other)	Glioblastoma Multiforme (GBM) Recurrent Glioblastoma Multiforme (GBM)	T cells	Yes, ex vivo	Lentivirus	Autologous	2024	University College London Hospitals NHS Foundation Trust, London
University College London	A CAR T Trial for Amyloid Light Chain Amyloidosis (AL Amyloid)	1010908 CPMS 65942 UCL/173497	Phase I	Recruiting	Metabolic (Other)	AL Amyloidosis	T cells	Yes, ex vivo	Lentivirus	Autologous	2025	University College London Hospitals NHS Foundation Trust, London Leeds Teaching Hospitals NHS Trust, Leeds University College London, 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	AdUP: AdNRGM; VDEPT + GMCSF in Locally Recurrent Prostate Cancer	MREC N°-12_SC_0660 UKCRN-13599 13599 UK-0208 RG_06-226 ISRCTN06254734	Phase I	Completed	Oncology (other)	Prostate Cancer	N/A	Yes, in vivo	Adenovirus	N/A	2013	Queen Elizabeth Hospital, Birmingham Institute of Cancer Studies, University of Birmingham, Birmingham
University of Birmingham	Selected Mesenchymal Stromal Cells to Reduce Inflammation in Patients with PSC and AIH (Merlin)	2016-000181-36 CPMS 33534 EudraCT-2016-000181-36 GDC30011048 GDCT0266794 IRAS-205917 ISRCTN73586959 RG-13-124 WP8	Phase II	In follow-up	Gastrointestinal (Liver)	Primary Sclerosing Cholangitis (PSC) and Autoimmune Hepatitis (AIH)	Mesenchymal stem/stromal cells	No	N/A	Allogeneic	2018	Queen Elizabeth Hospital, Birmingham

Sponsor	Title	Clinical Database Numbers	Trial Phase	Trial Status	Disease Area	Indication	Cell Type	Gene Modification / Gene Therapy	Type of Vector Used (if applicable)	Autologous/ Allogeneic	Year Trial Started	United Kingdom Site(s)
University of Birmingham	A Clinical Trial to See If a Mesenchymal Stem Cells Treatment Called Orbel-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 Upon Tyne Southampton General Hospital, Southampton
University of Birmingham	A Global Study of Novel Agents in Paediatric and Adolescent Relapsed and Refractory B-cell Non-Hodgkin Lymphoma (Glo-BNHL)	2021-004283-10 NCT05991388 RG_21-124 ITCC-100 1004701 EUCT-2024-510575-38-00 CTIS2024-510575-38-00	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 California	Efficacy and Safety of the Cryopreserved Formulation of OTL-101 in Subjects With ADA-SCID	CLIN2-09339 OTL-101-4 US-1562 EudraCT-2017-001275-23	Phase I/II	Completed	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 University College London, London
University of California	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 (ADA) Deficiency Related SCID	CD34 and/or CD133 stem cells	Yes, ex vivo	Lentivirus	Autologous	2019	Great Ormond Street Hospital for Children NHS Foundation Trust, London
University of Cambridge	TRANSEURO Open Label Transplant Study in Parkinson's Disease	FP7-242003 FOX-004409 242003 ISSN-1078-8956	Phase I	Completed	Neurological	Parkinson's Disease	Neural	No	N/A	Allogeneic	2012	Cambridge Cognition LTD, Cambridge Cardiff University, Cardiff Imperial College London, London University College London Hospital, London
University of Edinburgh NHS Lothian	Cell Therapy for Acute Liver Injury Trial	1005258 AC22087 IRAS 1005258 EudraCT-2022-002584-29 GDC50012752 GDCT0469986	Phase I	Recruiting	Gastrointestinal (Liver)	Acute Liver Injury	Monocytes	No	N/A	Allogeneic	2022	Edinburgh Royal Infirmary, Edinburgh Royal Victoria Infirmary, Newcastle Upon Tyne NHS Lothian, Edinburgh
University of Edinburgh NHS Lothian Academic and Clinical Central Office for Research and Development	Macrophage Therapy for Liver Cirrhosis (MATCH)	MATCH0.1 ISRCTN10368050 2015-000963-15 MR/M007588/1 MATCH01 IRAS 184291	Phase I/II	Completed	Gastrointestinal (Liver)	Liver cirrhosis	Monocytes	No	N/A	Autologous	2016	Edinburgh Royal Infirmary, Edinburgh Ninewells Hospital, Dundee Glasgow Royal Infirmary, Glasgow

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 Leeds	Tibial Fracture - Platelet-rich Plasma and Bone Marrow Concentrate (T-PAC)	226136	Phase II	In follow-up	Musculoskeletal	Tibial Diaphyseal Fracture	Bone marrow mononuclear cells	No	N/A	Autologous	2018	Leeds General Infirmary, Leeds Hull Royal Infirmary, Hull
University of Leeds	The Evaluation of Safety and Biological Effects of Cancer-killing Virus Vaccine given to Patients with Advanced Brain Tumours	2020-003405-59 CO20/134663 IRAS 283902 283902	Phase I	Recruiting	Oncology (Other)	Advanced Brain Tumours	N/A	No	Oncolytic Virus	N/A	2020	Leeds Teaching Hospitals NHS Trust, Leeds
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	Churchill Hospital, Oxford University Hospitals NHS Foundation Trust, Oxford
NIHR Evaluation Trials and Studies Coordinating Centre	REP1 Gene Replacement Therapy for Choroideremia (REGENERATE)	REGEN2015 UKCRN-19780 EudraCT-2015-001383-18 ISRCTN15602229 19780 IRAS-179453	Phase II	Completed	Ophthalmology	Choroideremia	N/A	Yes, <i>in vivo</i>	AAV	N/A	2016	Moorfields Eye Hospital NHS Foundation Trust, London Oxford University Hospitals NHS Foundation Trust, Oxford
Vertex Pharmaceuticals Inc	A Safety, Tolerability, and Efficacy Study of VX-264 in Participants with Type 1 Diabetes	VX22-264-101 NCT05791201 SNCTP000005450 BASEC2022-D0127 2024-515583-32-00 EUCT-2024-515583-32-00 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 University Hospitals NHS Foundation Trust, Oxford Royal Victoria Infirmary, Newcastle Upon Tyne
Vertex Pharmaceuticals Inc	A Safety and Efficacy Study Evaluating CTX001 in Participants with Transfusion-dependent β -Thalassemia	CTX001-111 EUCT-2024-516894-57-00 EudraCT-2017-003351-38 NCI-2020-01001 NCT03655678 Study 111 XX-0076	Phase I/II	In follow-up	Haematological	Transfusion-Dependent β Thalassemia	CD34 and/or CD133 stem cells	Yes, <i>ex vivo</i>	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 EudraCT-2018-001320-19 EUCT-2024-516067-83-00	Phase II/III	Completed	Haematological	Severe Sickle Cell Disease (SCD)	CD34 and/or CD133 stem cells	Yes, <i>ex vivo</i>	Non-viral	Autologous	2018	Imperial College Healthcare NHS Trust, London Barts Health NHS Trust, London

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 Long-term Follow-up Study in Participants Who Received CTX001	2018-002935-88 (EudraCT Number) 2024-512654-19-00 EUCT-2024-512654-19-00 EudraCT-2018-002935-88 study 131 VX18-CTX001-131 2024-512654-19-00 (Other Identifier) (OTHER: EU CT Number) 018143 255036	Phase III	Recruiting	Haematological	Severe Sickle Cell Disease (SCD)	CD34 and/or CD133 stem cells	Yes, <i>ex vivo</i>	Non-viral	Autologous	2021	Hammersmith Hospital, London Imperial College Healthcare NHS Trust, London University College London Hospitals NHS Foundation Trust, London
Vertex Pharmaceuticals Inc	A Safety, Tolerability, and Efficacy Study of VX-880 in Participants With Type 1 Diabetes	NCT04786262 VX20-880-101 EudraCT-2022-002292-11 SNCTP000005336 BASEC2022-01879 2022-002292-11-D 2022-002292-11-EN 2022-01879 2022-002292-11-DE 2024-513929-23-00 EUCT-2024-513929-23-00 VX22-880-101 NCI-2021-09777 FORWARD-101 1006463 IRAS-1006463	Phase II/III	Recruiting	Metabolic (Diabetes)	Transfusion-Dependent β -Thalassemia	Pancreatic islets	No	N/A	Allogeneic	2021	Churchill Hospital, Oxford University Hospitals NHS Foundation Trust, Oxford Royal Infirmary of Edinburgh, Edinburgh The Newcastle Upon Tyne Hospitals NHS Foundation Trust, Newcastle Upon Tyne King's College Hospital NHS Foundation Trust, London
Vertex Pharmaceuticals Inc	Evaluation of Safety and Efficacy of CTX001 in Pediatric Participants With Transfusion-Dependent β -Thalassemia (TDT)	VX21-CTX001-141 NCT05356195 2021-002172-39 (EudraCT Number) P/549/2021 2024-513349-35-00 EUCT-2024-513349-35-00 IND Number: 018143 018143	Phase III	Recruiting	Haematological	Diabetes	CD34 and/or CD133 stem cells	Yes, <i>ex vivo</i>	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)	VX21-CTX001-151 NCT05329649 study 151 2021-002173-26 (EudraCT Number) P/548/2021 2024-513978-22-00 EUCT-2024-513978-22-00 018143 IND 018143	Phase III	Recruiting	Haematological	Transfusion-Dependent β -Thalassemia	CD34 and/or CD133 stem cells	Yes, <i>ex vivo</i>	Non-viral	Autologous	2022	St Mary's Hospital, London
Vertex Pharmaceuticals Inc	A Phase I/II Study of VX-522 in Participants with Cystic Fibrosis (CF)	NCT05668741 VX21-522-001 2022-000726-25 (EudraCT Number) EUCT-2023-504786-23-00 2023-504786-23-00 (Other Identifier) (OTHER: EU CT Number) IRAS-1005878 1005878 EudraCT-2022-000726-25	Phase I/II	Recruiting	Respiratory	Severe Sickle Cell Disease (SCD)	N/A	Yes, <i>in vivo</i>	Non-viral	N/A	2023	Royal Papworth Hospital NHS Foundation Trust, Cambridge Queen Elizabeth University Hospital, Glasgow Royal Brompton and Harefield Hospitals, London Wythenshawe Hospital, Manchester University Hospital Llandough, Penarth University Hospital Southampton NHS Foundation Trust, Southampton

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)
Verve Therapeutics Inc	A Study of VERVE-101 in Patients With Familial Hypercholesterolemia and Cardiovascular Disease	NCT05398029 VT-1001 GDC30031492 GDCT0467180 IRAS-1005362 2022-000749-33	Phase I	Completed	Metabolic (Other)	Cystic Fibrosis	N/A	Yes, <i>in vivo</i>	Non-viral	N/A	2024	Unconfirmed UK Sites Clinical Study Centre, London
Verve Therapeutics Inc	Phase Ib Study of VERVE-201 in Patients with Refractory Hyperlipidemia	NCT06451770 VT-20101 DOH-27-122024-4794	Phase I	Recruiting	Metabolic (Other)	Hypercholesterolemia, Homozygous Familial Hypercholesterolemia (HoFH), Hyperlipidemia	N/A	Yes, <i>in vivo</i>	Non-viral	N/A	2024	Clinical Study Centre, Manchester
Verve Therapeutics Inc	A Study of VERVE-102 in Patients with Familial Hypercholesterolemia or Premature Coronary Artery Disease	NCT06164730 VT-10201	Phase I	Recruiting	Cardiovascular	Heterozygous familial hypercholesterolemia Coronary Artery Disease	N/A	Yes, <i>in vivo</i>	Non-viral	N/A	2024	Clinical Study Site, London Clinical Study Site, Manchester
Vivet Therapeutics SAS	A Phase I/II Study of VTX-801 in Adult Patients with Wilson's Disease (GATEWAY)	NCT04537377 VTX-801_CLN_001 2020-000963-22 EudraCT-2020-000963-22 EUCT-2023-509998-23-00 2023-509998-23-00 U1111-1301-8786	Phase I/II	Completed	Metabolic (Other)	Wilson's Disease	N/A	Yes, <i>in vivo</i>	AAV	N/A	2021	Royal Surrey County Hospital, Guildford