Cell and Gene Therapy Catapult UK Clinical Trials Database 2017

Name of Sponsor	Title	Project Summary	Clinical Database Numbers	Lead Institution/ Company and Collaborator Partners	United Kingdom Site(s)	Clinical Trial Status	Trial Phase	Year Trial Started	Recruitment Target	Cell Type	Cell Source	Gene Modification/ Gene	If applicable, type of virus vector used	Autologous/ Allogeneic	Disease Area	Indication	Contact
Cell Medica Ltd.	A Phase I/II clinical trial to investigate the safety of adenovirus-specific T-cells given to high-risk paediatric patients post allogeneic haematopoietic stem cell transplant (HSCT) to treat reactivation of adenovirus (ASPIRE trial).	Adoptive T cell therapy for the reconstitution of immunity to adenovirus (ADV) in paediatric patients following bone marrow transplantation	2011-001788-36	Cell Medica (3 UK Sites)	Great Ormond Street Hospital London, Royal Manchester Children's Hospital, Royal Victoria Infirmary	6- Closed	Phase I/II	2012	15 treated patients	T cells	Blood	No		Allogeneic	Cancer (Haematology)	ADV in paediatric patients following bone marrow transplantation	Shannon Inman, Cell Medica (shannon.inman@cellmedica.co.uk)
Cell and Gene Therapy Catapult Ltd	WT1 TCR Gene Therapy for Leukaemia: A Phase I/II Safety and Toxicity Study (WT1 TCR-001)	WT1 TCR gene therapy for leukaemia: a phase I/II safety and toxicity study (WT1 TCR-001)	2006-004950-25 NCT01621724	University College London	Queen Elizabeth Hospital University Hospitals Bristol NHS Foundation Trust University College London Hospitals NHS Trust	3- Recruiting	Phase I/II	2012	18	T cells	Blood	Yes ex-vivo	Gamma-retrovirus	Autologous	Cancer (Haematology)	Acute myloid leukaemia; chronic myloid leukaemia	Sara Marques Sara.Marques@ct.catapult.org.uk Contact: Emma Morris, Dr e.morris@ucl.ac.uk
The Christie NHS Foundation Trust	A Phase II Trial to Assess the Activity of NY-ESO-1 Targeted T Cells in Advanced Oesophagogastric Cancer (ATTACK-OG).	This is a trial of adoptive T cell therapy using the patient's own T cells, genetically engineered to target the tumour associated antigen NY-ESO-1 (New York esophageal squamous cell carcinoma 1).	NCT01795976; UK CRN 14133; 83343031	Christie Hospital NHS Foundation Trust Erasmus Medical Center Ospedale San Raffaele University College London Hospitals Karolinska University Hospital The Netherlands Cancer Institute	The Christie NHS Foundation Trust Manchester	4- Suspended	Phase II	2013	28	T cells	Blood	Yes ex-vivo	Lentiviral vector expressing the tumour antigen NY-ESO-1	Autologous	Cancer	Oesophagogastric cancer	Prof Robert Hawkins (The Christie NHS Foundation Trust) / Ryan Guest (Cellular Therapeutics Ltd)
Great Ormond Street Hospital NHS Trust / University College London	Gene therapy for SCID-X1 using a self-inactivating (SIN) gammaretroviral vector.	Gene therapy for SCID-X1. Autologous haematopoietic stem cells transplanted after modification with a self-inactivating gammaretroviral vector expressing the human common cytokine receptor gamma-chain gene	2007-000684-16	Great Ormond Street Hospital, London	Great Ormond Street Hospital, London	5- In follow-up	Phase I/II	2011	10	CD34 and/or CD133 stem cells	Other	Yes ex-vivo	Self-inactivating (SIN) Gammaretrovirus	Autologous	Inflammatory and immune system	X-linked severe combined immunodeficiency	Havinder Hara Clinical Project Manager UCL Institute of Child Health London h.hara@ucl.ac.uk
Great Ormond Street Hospital NHS Trust	Phase I/II, non-controlled, open-label, non-randomised, single-centre trial to assess the safety and efficacy of EF1αS-ADA lentiviral vector mediated gene modification of autologus CD34+ cells from ADA-deficient individuals	Lentiviral gene therapy for ADA-SCID. Autologous haematopoietic stem cells transplanted after modification with a lentiviral vector expressing the human ADA gene	2010-024253-36; NCT01380990	Great Ormond Street Hospital, London	Great Ormond Street Hospital, London	5- In follow-up	Phase I/II	2012	10	CD34 and/or CD133 stem cells	Blood	Yes ex-vivo	Lentiviral vector	Autologous	Inflammatory and immune system	Adenosine Deaminase Deficiency	Havinder Hara Clinical Project Manager UCL Institute of Child Health London h.hara@ucl.ac.uk
UK Stem Cell Foundation / Heart Cells Foundation	Randomised Controlled Clinical Trial of the Use of Autologous Bone Marrow Derived Progenitor Cells to Salvage Myocardium in Patients With Acute Anterior Myocardial Infarction (REGEN-AMI)	Autologous bone marrow derived mononuclear cells for acute myocardial infarction. Combines stem cell delivery with primary angioplasty within 5 hours post event	NCT00765453	Barts Health NHS Trust, Queen Mary University of London, University College London	London Chest Hospital, Barts and The London NHS Trust, London The Heart Hosptial, UCLH Foundation Trust, London The Royal Free Hospital,	5- In follow-up	Phase II	2007	100	Bone marrow mononuclear cells	Bone marrow	No		Autologous	Cardiovascular	Acute myocardial infarction	Professor Anthony Mathur, William Harvey Research Institute, Queen Mary University (a.mathur@qmul.ac.uk)
Queen Mary University of London	The effect of intracoronary reinfusion of bone marrow-derived mononuclear cells (BM-MNC) on all cause-mortality in acute myocardial infarction	Autologous bone marrow derived mononuclear cells for patients with impaired LV function post myocardial infarction, delivered via intracoronary injection	UK CRN15079; NCT01569178	Barts Health NHS Trust, Queen Mary University of London	New Cross Hospital, Wolverhampton Queen Mary University of London, London University College London, London	3- Recruiting	Phase III	2011	350-400	Bone marrow mononuclear cells	Bone marrow	No		Autologous	Cardiovascular	Acute myocardial infarction	Professor Anthony Mathur, William Harvey Research Institute, Queen Mary University (a.mathur@qmul.ac.uk)
University of Cambridge	An Open Label Study to Assess the Safety and Efficacy of Neural Allo-Transplantation With Fetal Ventral Mesencephalic Tissue in Patients With Parkinson's Disease	Fetal brain tissue transplant for Parkinson's disease (TRANSEURO: An Innovative Approach for the Treatment of Parkinson's Disease)	NCT01898390	Univeristy of Cambridge Lund University Cardiff University Imperial College London University College London Univesity Hospital Freiburg Life Science Governance Institute Assistance Publique - Hopitaux de Paris Institut National de la	Cardiff University Imperial College London University College London University of Cambridge	5- In follow-up	Phase I/II	2012	40: 20 transplanted patients, 20 controls	Neural	Tissue	No		Allogeneic	Neurological	Parkinson's disease	Natalie Valle Guzman, University of Cambridge, Transeuro trial manager
ReNeuron Limited, UK	A Phase I Safety Trial of CTX0E03 Drug Product Delivered Intracranially in the Treatment of Patients With Stable Ischemic Stroke	CTX stem cells for the treatment of stroke disability (PISCES)	EudraCT: 2008-000696-19 ClinTrials: NCT01151124	Queen Elizabeth University Hospital	Glasgow Southern General Hospital	5- In follow-up	Phase I	2010	12	Neural	Tissue	No		Allogeneic	Neurological	Stroke disability	Dr John Sinden, ReNeuron Ltd.: info@reneuron.com

Cell and Gene Therapy Catapult UK Clinical Trials Database 2017

Name of Sponsor	Title	Project Summary	Clinical Database Numbers	Lead Institution/ Company and Collaborator Partners	United Kingdom Site(s)	Clinical Trial Status	Trial Phase	Year Trial Started	Recruitment Target	Cell Type	Cell Source	Gene Modification/ Gene	If applicable, type of virus vector used	Autologous/ Allogeneic	Disease Area	Indication	Contact
ReNeuron Limited, UK	A Phase II Efficacy Study of Intracerebral CTX0E03 DP in Patients with Stable Paresis of the Arm Following an Ischaemic Stroke	CTX stem cells for the treatment of stroke disability (PISCES II)	EudraCT: 2012-003482-18 ClinTrials: NCT02117635	Queen Elizabeth University Hospital	Queen Elizabeth Hospital, Birmingham NHS Southern General Hospital, Glasgow King's College Hospital, London Univesity College London Hospital Royal Victoria Infimary, Newcastle	5- In follow-up	Phase II	2014	21	Neural	Tissue	No		Allogeneic	Neurological	Stroke disability	Dr John Sinden, ReNeuron Ltd.: info@reneuron.com
ReNeuron Limited, UK	A Phase I Ascending Dose Safety Study Of Intramuscular CTX0E03 In Patients With Lower Limb Ischaemia	CTX stem cells for the treatment of Lower Limb Ischaemia (Safety study)	EudraCT: 2011-005810-13 ClinTrials: NCT01916369	Ninewells Hospital, Dundee	Ninewells Hospital, Dundee	5- In follow-up	Phase I	2014	9	Neural	Tissue	No		Allogeneic	Cardiovascular	Peripheral Arterial Disease-lower limb ischaemia	Dr John Sinden, ReNeuron Ltd.: info@reneuron.com
The European Blood and Marrow Transplant Group	Autologous stem cell transplantation international Crohn's disease trial	Autologous CD34+ haematopoietic cells for Crohn's disease	2005-003337-40 ; ISRT39133198 ; UK CRN 7107	European Group for Blood and Marrow Transplantation (EBMT)	Nottingham University Hospital	6- Closed	Phase II/III	2006	45	CD34 and/or CD133 stem cells	Bone marrow	No		Autologous	Oral and Gastrointestinal	Crohn's disease	Prof Hawkey, NDDC, West Block, E Floor, University Hospital, QMC, Nottingham NG7 2UH (ci.hawkey@nottingham
Newcastle upon Tyne Hospitals NHS Foundation Trust	Treatment of LSCD using cultured limbal epithelium expanded ALSC	Autologous cultured human limbal epithelium for limbal stem cell deficiency (ophthalmology)	2011-000608-16 ; 51772481 ; UK CRN 11185	Newcastle University	N/A	5- In follow-up	Phase II	2012	24	Corneal	Tissue	No		Autologous	Eye	Limbal stem cell deficiency	Professor Francisco C Figueiredo, Newcastle University, UK
University of Newcastle upon Tyne	Biomedical / psychosocial islet cell transplant outcomes	Biomedical and psychosocial outcomes of islet transplantation within the NHS clinical programme	UK CRN 4166	Newcastle University	N/A	3- Recruiting	Phase III	2007	100	Pancreatic islets	Other	No		Allogeneic	Diabetes	Type 1 diabetes complicated by recurrent severe hypoglycaemia	Prof James Shaw, Institute of Cellular Medicine, Newcastle University
The Robert Jones and Agnes Hunt Orthopaedic Hospital NHS Foundation Trust	Autologous Cell Therapy for Osteoarthritis: An evaluation of the safety and efficacy of autologous transplantation of articular chondrocytes and/or bone marrow-derived stromal cells to repair chondral/osteochondral lesions of the knee (ASCOT).	The principal research question of this trial is to find out if treatment with either a patient's own cartilage cells (selected and culture expanded chondrocytes), or bone marrow-derived stromal cells (containing selected and culture expandedstem cells), or a combination of the two cell types, give a different clinical outcome, in terms of knee function, for patients with early osteoarthritis of the knee.	2010-022072-31	The Robert Jones and Agnes Hunt Orthopaedic Hospital NHS Foundation Trust	The Robert Jones and Agnes Hunt Orthopaedic Hospital NHS Foundation Trust	3- Recruiting	Phase II	2013	114	Mesenchymal stem/stromal cells	Bone marrow	No		Autologous	Bone and cartilage	Osteochondral defects of the knee (early osteoarthritis)	Prof James Richardson; Dr Johanna Wales
Azellon Ltd, UK	A Prospective Open-Label Study to Evaluate the Safety of Cell Bandage (Mesenchymal Stem Cells) in the Treatment of Meniscal Tears	Autologous mesenchymal stem cells (MSCs) for knee meniscal repair. MSCs grown on biological scaffold for 2 weeks then surgically implanted	2010-024162-22	Azellon Cell Therapeutics	N/A	3- Recruiting	Phase I/II	2012	10	Mesenchymal stem/stromal cells	Bone marrow	No		Autologous	Bone and cartilage	Knee meniscus repair	Professor Anthony Hollander, (CSO at Azellon); Univeristy of Bristol ()
Newcastle upon Tyne Hospitals NHS Foundation Trust	Autologous Tolerogenic Dendritic Cells for Rheumatoid and Inflammatory Arthritis	Patients with inflammatory arthritis with active involvement of a knee joint undergo leukapheresis. Monocytes are positively selected and differentiated into tolerogenic dendritic cells over the course of 7 days. The tolerogenic dendritic dendritic cells are then arthroscopically injected into the inflamed knee following saline wash-out. Primary outcomes are safety and tolerability. Biomarkers will be measured in synovial mambrane biopsies and peripheral blood (baseline and +14 days). In this ascending dose study we will study one, three and ten million tolerogenic DCs (3 patients per cohort) and there is also a placebo cohort who receive saline washout only. Follow-up is for thirteen weeks post administration of tolerogenic DCs. The main study has completed but we	NCT01352858; 87426082 ; UK CRN 12108	Arthritis Research UK Newcastle-upon-Tyne Hospitals NHS Trust	Newcastle RVI	3- Recruiting	Phase I	2011	12 plus 3 in extension study	Antigen presenting cells	Blood	No		Autologous	Inflammatory and immune system	Rheumatoid and Inflammatory Arthritis	Prof John Isaacs Newcastle University Institute of Cellular Medicine Framlington Place Newcastle Upon Tyne Tyne and Wear NE1 7RU UNITED KINGDOM
University College London	CMV TCR Gene Therapy: A Phase I Safety, Toxicity and Feasibility Study of Adoptive Immunotherapy with CMV TCR-transduced Donor-derived T cells for Recipients of Allogeneic Haematopoietic Stem Cell Transplantation	CMV TCR Gene Therapy: A Phase I Safety, Toxicity and Feasibility Study of Adoptive Immunotherapy with CMV TCR-transduced Donor-derived T cells for Recipients of Allogeneic Haematopoietic Stem Cell Transplantation	UK CRN 12518 ; 2008-006649-18	UCL	N/A	3- Recruiting	Phase I	2013	10	T cells	Other	Yes ex-vivo	Retroviral vector	Allogeneic	Cancer (Haematology)	CMV seronegative HSCT donors & CMV seropositive HSCT recipients	Dr Emma Morris e.morris@ucl.ac.uk or Rachel Richardson University College London Institute of Immunity and Transplantation Rowland Hill Street Hampstead London NW3 2PF UNITED KINGDOM
University College London	Immunotherapy with CD25/71 Allodepleted T-cells (ICAT)	Adoptive Immunotherapy with CD25/71 allodepleted donor T-cells to improve immunity after unrelated donor stem cell transplant (ICAT)	UK CRN14779 ; NCT01827579	CR UK and UCL Cancer Trials Centre Medical Research Council	Manchester Royal Infirmary University College London Hospital, London	3- Recruiting	Phase II	2014	24	T cells	Blood	No		Allogeneic	Cancer (Haematology)	Haematological Malignancies	ICAT trial coordinator Cancer Research UK & UCL Cancer Trials Centre 90 Tottenham Court Road London W1T 4TJ UNITED KINGDOM Tel: 0207 679 9327

Cell and Gene Therapy Catapult UK Clinical Trials Database 2017

Name of Sponsor	Title	Project Summary	Clinical Database Numbers	Lead Institution/ Company and Collaborator Partners	United Kingdom Site(s)	Clinical Trial Status	Trial Phase	Year Trial Started	Recruitment Target	Cell Type	Cell Source	Gene Modification/ Gene	If applicable, type of virus vector used	Autologous/ Allogeneic	Disease Area	Indication	Contact
King's College London	Phase I Trial: T4 Immunotherapy of Head and Neck Cancer	Patients with locally advanced/ recurrent head and neck cancer will receive autologous gene-modified by intratumoral injection in this Phase 1 dose escalation study. T-cells will be engineered to co-express a broadly reactive ErbB-targeted CAR with a chimeric cytokine receptor that allows ex-vivo expansion of cell products using IL-4.	NCT01818323	Guy's and St Thomas' NHS Foundation Trust	Guy's Hospital, London	3- Recruiting	Phase I	2015	30	T cells	Blood	Yes ex-vivo	Retroviral vector	Autologous	Cancer	Locally advanced/ recurrent disease for which no suitable alternative therapy is available	John Maher King'sCollege London, john.maher@kcl.ac.uk
Cardiff University	Safety and feasibility of neural transplantation in early to moderate Huntington's disease in the UK.	Safety and feasibility of neural transplantation in early to moderate Huntington's disease in the UK.	UKCRN 3827	Cardiff University	N/A	2- In set-up	Phase I	2014	60	Neural	Other	No		Allogeneic	Neurological	Neurological	Prof Anne Rosser, The Brain Repair Group, School of Biosciences, Cardiff University, Museum Avenue, Cardiff CF10 3AX, South Wales, U.K.
Guy's and St Thomas' NHS Foundation Trust	Safety and Efficacy Study of Regulatory T Cell Therapy in Liver Transplant Patients (ThRIL)	This is a clinical trial in patients undergoing liver transplantation. Research has shown that regulatory T-cells can induce tolerance to the graft in laboratory animals that have undergone organ transplantation. In this study, liver recipients will receive a single infusion of TR002, a cell therapy product that consists of regulatory T-cells that are grown and purified from the patients' own blood. The trial aims to explore the feasibility, safety, and efficacy of TR002 as add-on immunosuppressive treatment in the context of liver transplantation.	NCT02166177, UK CRN 16775	Kings College Hospital	N/A	3- Recruiting	Phase I/II	2014	26	T cells	Blood	No		Autologous	Inflammatory and immune system	End-Stage Liver Disease	Alberto Sanchez-Fueyo, MD, PhD Gavin Whitehouse, BM, MRCP(UK)
University College, London	Clinical Trial of Stem Cell Based Tissue Engineered Laryngeal Implants (RegenVOX)	This study aims to test a new groundbreaking treatment for narrowing of the voicebox and upper windpipe, which can be due to injury, inflammatory disease or cancer treatment. The new treatment tested by this study is an implant that will partially replace the voicebox or upper windpipe in order to cure the narrowing. The implant is based on a human donor voicebox or windpipe that has been processed with detergents and enzymes in order to remove all the cells from the donor, leaving a 'scaffold' of connective tissue. The patient's own stem cells are removed from the bone marrow, then are grown on the scaffold in the laboratory. These cells will form the cartilage in the wall of the scaffold. A split skin graft from the patient may be needed to line the inside of the implant. Once these cells have attached and started to grow on the scaffold, it is ready to be implanted into the patient, and an operation is performed which occurs in two separate stages. The final stage of the operation involves removing the narrow	NCT01977911	University College, London	University College London Hospital, London	3- Recruiting	Phase I/II	2015	10	Bone marrow mononuclear cells	Bone marrow	No		Autologous	Respiratory	Ear, Nose and Throat	Martin Birchall and Steve Bloor
Cell Medica Inc	A Phase 2 Single Arm Study to Investigate the Efficacy of Autologous EBV-specific T-cells for the Treatment of Patients With Aggressive EBV Positive Extranodal NK/T-cell Lymphoma (ENKTCL)	Autologous EBV specific T-cells for treatment of EBV+ve lymphomas (CITADEL Study)	NCT01948180	Cell Medica (24 clinical sites, US, UK, Fr, De and SK)	University College London Hospital, London The Christie Clinic, Manchester	3- Recruiting	Phase II	2015	35	T cells	Blood	No		Autologous	Cancer (Haematology)	NK/T cell lymphoma	Shannon Inman, Cell Medica (shannon.inman@cellmedica.co.uk)
Guy's and St Thomas' NHS Foundation Trust	The ONE Study UK Treg Trial (ONETreg1)	A study to assess cell therapy as a treatment to prevent kidney transplant rejection. The trial will involve purification of naturally occurring regulatory T cells (nTregs) from living-donor renal transplant recipients. The cells will then be grown in the laboratory and re-infused into the patient five days after the kidney transplant. This trial is part of an international European Union funded consortium aimed at evaluating cellular immunotherapy in solid organ transplantation (The ONE Study). It is anticipated that immune regulation induced by nTreg therapy can eventually be used to reduce the need for conventional immunosuppression in transplant recipients.	NCT02129881	King's College London	Guy's Hospital Recruiting London, The Oxford Transplant Centre - Churchill Hospital	5- In follow-up	Phase I/II	2014	12	T cells	Blood	No		Autologous	Inflammatory and immune system	End-stage kidney disease	Dr Rachel Hilton BMBCh PhD
Cell and Gene Therapy Catapult Ltd	A single arm Phase I/II study of the safety and efficacy of gene-modified WT1 TCR therapy in patients with myelodysplastic syndrome (MDS) or acute myeloid leukaemia (AML) who have failed to achieve or maintain an IWG defined response following hypomethylating agent therapy.	This is a Phase I/II trial to determine safety, clinical efficacy and feasibility of a gene-modified WT1 TCR therapy in patients with myelodysplastic syndrome (MDS) and acute myeloid leukaemia (AML). Patient's white blood cells (T cells) will be modified by transferring a gene which enables them to make a new T cell receptor (TCR) that can recognize fragments of a protein called WT1 (Wilms' tumour 1) which is present at abnormally high levels on the surface of myelodysplastic and leukaemic cells. In this trial, approximately 25 participants with an Human Leukocyte Antigen A2 (HLA-A*0201) tissue type who have failed to achieve or maintain an IWG defined response following hypomethylating agent therapy will be recruited.	2014-003111-10 NCT02550535	University College Hospital London	Aberdeen Royal Infirmary Recruiting Aberdeen, United Kingdom University Hospitals Bristol NHS Foundation Trust Recruiting Bristol, Western General Hospital, Edinburgh The Leeds Teaching Hospitals NHS Trust, Leeds University College London Hospitals NHS Trust, London	3- Recruiting	Phase I/II	2015	25	T cells	Blood	Yes ex-vivo	Gamma-retrovirus	Autologous	Cancer (Haematology)	Myeloidysplastic Syndrome and Acute Myeloid Leukaemia	Jacqueline Barry jacqueline.barryr@ct.catapult.org.uk Dominic Bowers dominic.bowers@ct.catapult.org.uk
Cell and Gene Therapy Catapult Ltd	A Phase I Open-label Study to Assess the Safety, Tolerability and Potential Efficacy of a Novel Tracheal Replacement Consisting of a Tissue-engineered Decellularised Tracheal Scaffold With Seeded Autologous Mesenchymal Cells in Subjects With Severe Tracheal Stenosis or Malacia	This is a phase I study to evaluate the safety, tolerability and potential efficacy of a novel tracheal replacement therapy using cadaveric de-cellularised tracheal scaffold and patients' own mesenchymal cells isolated from a sample of their bone marrow in patients' who suffer from severe tracheal malacia or stenosis and for whom conventional therapies are no longer adequate. A total of 4 patients will be treated during the course of this study.	2015-002108-10 NCT02949414	University College London Videregen	Royal Nose Throat and Ear Institute	2- In set-up	Phase I	Expected 2015	4	Mesenchymal stem/stromal cells	Bone marrow	No		Autologous	Respiratory	Tracheal Stenosis and Tracheomalacia	Martin Birchall University College London Jacqueline Barry Cell and Gene Therapy Catapult

Cell and Gene Therapy Catapult UK Clinical Trials Database 2017

Name of Sponsor	Title	Project Summary	Clinical Database Numbers	Lead Institution/ Company and Collaborator Partners	United Kingdom Site(s)	Clinical Trial Status	Trial Phase	Year Trial Started	Recruitment Target	Cell Type	Cell Source	Gene Modification/ Gene	If applicable, type of virus vector used	Autologous/ Allogeneic	Disease Area	Indication	Contact
Athersys, Inc, USA	A Phase 1/2 Study to Assess the Safety and Efficacy of MultiStem® Therapy in Subjects with Acute Respiratory Distress Syndrome	A Phase 1/2 Study to Assess the Safety and Efficacy of MultiStem® Therapy in Subjects with Acute Respiratory Distress Syndrome	2015-001586-96	University College London Cell Therapy Catapult	University College London Hospital, London St Georges Hospital, London Queen Elizabeth Hospital, Birmingham John Radcliffe Hospital, Oxford Addenbrookes Hospital, Cambridge Wythenshawe Hospital, Manchester Manchester Royal Infirmary, Manchester	3- Recruiting	Phase I/II	2015	40	Mesenchymal stem/stromal cells	Bone marrow	No		Allogeneic	Respiratory	Acute Respiratory Distress Syndrome	Jacqueline Barry Cell and Gene Therapy Catapult 12th Floor Tower Wing Guy's Hospital Great Maze Pond SE1 9RT
King's College London and Guy's & St Thomas' NHS Foundation Trust	Phase I study of COL7A1 gene-modified autologous fibroblasts in adults with recessive dystrophic epidermolysis bullosa.	Phase I study to evaluate whether intradermal injections of COL7A1 gene-modified autologous fibroblasts are safe in adults with recessive dystrophic epidermolysis bullosa.	NCT02493816	King's College London	Guy's and St Thomas' NHS Foundation Trust	3- Recruiting	Phase I	2015	5 to 10	Fibroblasts	Tissue	Yes ex-vivo	Lentiviral vector	Autologous	Skin	Recessive dystrophic epidermolysis bullosa	Professor John A. McGrath Guy's Hospital Great Maze Pond London SE1 9RT UNITED KINGDOM Tel: 02071886409 john.mcgrathf@kcl.ac.uk
Innovacell Biotechnologie AG, Austria	Skeletal muscle-derived cell implantation for the treatment of fecal incontinence: a multicenter, randomized, double-blind, placebo-controlled, parallel-group, dose-finding clinical study	Ongoing clinical trial for clinical investigation of aSMDC therapy of FI with the research medicinal product ICEF15. Objective of the study is to find the optimal cell count for functional regeneration of the external anal sphincter. The study is planned as a multinational, multicenter, randomized, double-blind, placebo-controlled, parallel-group, clinical study. A maximum of 252 female and male patients with external anal sphincter weakness or sphincter damage suffering from FI will be investigated to achieve 207 evaluable datasets. Patients are randomized to one of three groups: cell dose 1, cell dose 2, placebo (which consists of cell-free medium). Observation period is 6 months post treatment. All patients perform electrical stimulation for a total of 8 weeks, 4 weeks after biopsy and prior to implantation and 4 weeks starting immediately after	2010-021463-32	ICTA company (CRO) / University College London Hospitals	N/A	5- In follow-up	Phase II	2013	252	Skeletal Muscle	Other	No		Autologous	Musculoskeletal	Faecal Incontinence	Dr. Rainer Marksteiner, Chief Executive Officer, Innovacell Biotechnologie AG, Mitterweg 24, 6020 Innsbruck, Austria
Cook MyoSite, USA	A Prospective Nonrandomized Study of Autologous Muscle Derived Cell (AMDC) Transplantation for Treatment of Fecal Incontinence	The aim of this clinical study is to investigate the safety and feasibility of Autologous Muscle Derived Cells (AMDC; a preparation of a patient's own cells) injection into the anal sphincter for treatment of patients with fecal incontinence.	NCT01600755	Royal Hospital of London, National Centre for Bowel Research & Surgical Innovation	N/A	3- Recruiting	Phase I/II	2012	50	Skeletal Muscle	Other	No		Autologous	Musculoskeletal	Faecal Incontinence	Yahira Baez-Santos, Travis Conley travis.conley@cookmedical.com
University College London	Autologous Stem Cells in Achilles Tendinopathy (ASCAT)	This study is looking at a new treatment, using the patient's own stem cells (the repair cells of the body), to see whether this can help reduce pain and promote healing of the Achilles tendon, without side effects.	NCT02064062	University College London Hospital	Royal National Orthopaedic Hospital	3- Recruiting	Phase II	2015	10-Jan	Mesenchymal stem/stromal cells	Other	No		Autologous	Musculoskeletal	Achilles Tendinopathy	Andrew Golberg Royal National Orthopaedic Hospital andy.goldberg@rnoh.nh
University College London	COBALT: Evaluation of CAR19 T-cells as an Optimal Bridge to Allogeneic Transplantation	The purpose of this study is to administer novel cluster of differentiation antigen 19 (CD19) specific Chimeric Antigen Receptor T-cells (CAR19 T-cells) to patients with relapsed or resistant Diffuse Large B Cell Lymphoma (DLBCL) to assess the safety and efficacy of this strategy as a bridge to allogeneic transplantation	NCT02431988	University College London Hospital	University College London Hospital, London	3- Recruiting	Phase I	2015	12	T cells	Blood	Yes ex-vivo	Lentiviral vector	Autologous	Cancer (Haematology)	Diffuse Large B-Cell Lymphoma	COBALT trial coordinator at ctc.cobalt@ucl.ac.uk
University College London	CARPALL: Immunotherapy with CD19 CAR redirected T-cells for high risk, relapsed paediatric CD19+ acute lymphoblastic leukaemia and other haematological malignancies.	The purpose of this study is to evaluate the safety, efficacy and duration of response of a novel cluster of differentiation antigen 19 (CD19) specific Chimeric Antigen Receptor T-cells (CD19CAR T-cells) to paediatric patients with high risk acute lymphoblastic leukaemia (ALL) and other haematological malignancies.	NCT02443831	Leading: 1-University College London Institute of Child Health/Great Ormond St Hospital. Collaborators: 2-University College London Hospitals 3-Royal Manchester Children's Hospital	Great Ormond Street Hospital for Children London, United Kingdom, WC1N 3JH University College London Hospital London, United Kingdom; Royal Manchester Children's Hospital	3- Recruiting	Phase I/II	2016	18	T cells	Blood	Yes ex-vivo	Lentiviral vector	Autologous	Cancer (Haematology)	Paediatric Acute Lymphoblastic Leukaemia and other haematological malignancies (e.g. Burkitt's lymphoma)	CARPALL trial coordinator at ctc.carpall@ucl.ac.uk
The University of Edinburgh	Macrophage Therapy for Liver Cirrhosis (MATCH)	A multicentre, phase I/II trial of repeated infusions of autologous CD14+ monocyte-derived macrophages in patients with liver cirrhosis	2015-000963-15	The University of Edinburgh, SNBTS, NHS Lothian, Cell Therapy Catapult	Edinburgh Royal Infirmary	3- Recruiting	Phase I/II	2016	74	Other	Blood	No		Autologous	Liver	Advanced Liver Cirrhosis	Prof Stuart Forbes University of Edinburgh Centre for Regenerative Medicine MRC Edinburgh EH16 4TJ UNITED KINGDOM stuart.forbes@ed.ac.uk
IRCCS - Istituto di Ricerche Farmacologiche Mario Negri	Novel Stromal Cell Therapy for Diabetic Kidney Disease (NEPHSTROM)	A multicentre, phase 1 and 2 trial to investigate, primarily, the safety, feasibility and tolerability and, secondarily, the preliminary efficacy of an allogeneic bone marrow-derived Mesenchymal Stromal Cell (MSC) therapy (ORBCEL-M) in study subjects with type 2 diabetes (T2D) and progressive diabetic kidney disease (DKD).	NCT02585622 EudraCT: 2016-000661-23	Leiden University Medical Center, Leiden, The Netherlands NHS Blood and Transplant, Liverpool, UK, ASST Papa Giovanni XXIII Bergamo, Italy IRCCS - Istituto di Ricerche Farmacologiche Mario Negri, Bergamo, Italy National University of Ireland, Galway, Ireland,	Belfast Health and Social Care Trust - Belfast City Hospital Belfast, United Kingdom University Hospital Birmingham NHS Foundation Trust - Queen Elizabeth Medical Centre Birmingham, United Kingdom	2- In set-up	Phase I/II	2017	48	Mesenchymal stem/stromal cells	Bone marrow	No		Allogeneic	Renal and Urogenital	Diabetic kidney disease	Peter Maxwell, MD (Belfast City Hospital) Paul Cockwell, MD (Queen Elizabeth Medial Centre)

Cell and Gene Therapy Catapult UK Clinical Trials Database 2017

Name of Sponsor	Title	Project Summary	Clinical Database Numbers	Lead Institution/ Company and Collaborator Partners	United Kingdom Site(s)	Clinical Trial Status	Trial Phase	Year Trial Started	Recruitment Target	Cell Type	Cell Source	Gene Modification/ Gene	If applicable, type of virus vector used	Autologous/ Allogeneic	Disease Area	Indication	Contact
Kiadis Pharma, Netherlands	Safety and Efficacy of Two Doses of ATIR101, a T-lymphocyte Enriched Leukocyte Preparation Depleted of Host Alloreactive T-cells, in Patients With a Hematologic Malignancy Who Received a Hematopoietic Stem Cell Transplantation From a Haploidentical Donor	An Exploratory, Open-label, Multicenter Study to Evaluate the Safety and Efficacy of a Two-dose Regimen of ATIR101, a T-lymphocyte Enriched Leukocyte Preparation Depleted ex Vivo of Host Alloreactive T-cells (Using Photodynamic Treatment), in Patients With a Hematologic Malignancy, Who Received a CD34-selected Hematopoietic Stem Cell Transplantation From a Haploidentical Donor	NCT02500550	Kiadis Pharma, Netherlands	Heartlands Hospital Not yet recruiting Birmingham, United Kingdom, B9 5SS Hammersmith Hospital Recruiting London, United Kingdom, W12 ONN	3- Recruiting	Phase II	2015	15	CD34 and/or CD133 stem cells	Bone marrow	No		Allogeneic	Cancer (Haematology)	Acute Myeloid Leukaemia (AML), Acute Lymphoblastic Leukaemia (ALL) and Myelodysplastic Syndrome (MDS)	clinicaltrials@kiadis.com
CellProthera, France	EXpanded CELL ENdocardiac Transplantation (EXCELLENT)	A Multicentric Controlled Phase I / IIb Study Evaluating the Safety and the Efficacy of in Vitro Expanded Peripheral Blood CD34+ Stem Cells Output by the StemXpand® Automated Process, and Injected in Patients With an Acute Myocardial Infarction and a Left Ventricle Ejection Fraction (LVEF) Remaining Below or Equal to 45% After PTCA and Stent(s) Implantation Versus Standard of Care	NCT02669810	CellProthera, France	University of Edinburgh Leeds University & Leeds Teaching Hospitals NHS Trust Newcastle University	3- Recruiting	Phase I/II	2016	44	CD34 and/or CD133 stem cells	Bone marrow	No		Autologous	Cardiovascular	Acute Myocardial Infarction	Contact: Anthony Criquet, MD
Great Ormond Street Hospital for Children NHS Foundation Trust	Phase I Study of Ex-vivo Lentiviral Gene Therapy for the Inherited Skin Disease Netherton Syndrome	Netherton Syndrome is a serious skin disorder caused by damage in a gene called SPINK5. This gene controls the formation of a protein called LEKTI, which important for skin barrier function. The investigators have been developing a gene therapy approach using a disabled virus (vector) to carry a functional copy of the SPINK5 gene into skin stem cells. In this trial the investigators propose grafting of autologous epidermal sheets generated from genetically modified skin stem cells for the treatment of patients with Netherton Syndrome.	NCT01545323	Great Ormond Street Hospital for Children NHS Foundation Trust	Guy's and St Thomas NHS Trust, London Great Ormond Street Hospital for Children NHS Trust , London	3- Recruiting	Phase I	2014	5	Other	Tissue	Yes ex-vivo	Lentiviral vector	Autologous	Skin	Netherton Syndrome	Havinder Hara Senior Clinical Project Manager UCL Great Ormond Street Institute of Child Health Molecular and Cellular Immunology Section 30 Guildford Street London WC1N 1EH
Genethon	Phase I/II Clinical Trial of Haematopoietic Stem Cell Gene Therapy for the Wiskott-Aldrich Syndrome	This is a phase I/II study to evaluate the safety and efficacy of Hematopoietic Stem Cell gene therapy for the Wiskott-Aldrich Syndrome	NCT01347242	Great Ormond Street Hospital NHS Foundation Trust, London, UK UCL Institute of Child Health, London UK	Great Ormond Street Hospital Recruiting London, United Kingdom, WC1N 1EH Royal Free Hospital Recruiting London, United Kingdom, WC1N 1EH	3- Recruiting	Phase I/II	2011	5	CD34 and/or CD133 stem cells	Bone marrow	Yes ex-vivo	Lentiviral vector	Autologous	Inflammatory and immune system	Wiskott-Aldrich Syndrome (WAS)	Prof Adrian Thrasher UCL ICH
Great Ormond Street Hospital for Children NHS Foundation Trust	Gene Therapy for X-linked Severe Combined Immunodeficiency (SCID-X1)	X-linked severe combined immunodeficiency (SCID-X1) is an inherited disorder that results in failure of development of the immune system in boys. This trial aims to treat SCID-X1 patients usinga self-inactivating (SIN) gammaretroviral vector to replace the defective gene.	NCT01175239	Great Ormond Street Hospital NHS Foundation Trust, London, UK UCL Institute of Child Health, London UK	Great Ormond Street Hospital for Children NHS Trust London,	5- In follow-up	Phase I/II	2011	1	CD34 and/or CD133 stem cells	Bone marrow	Yes ex-vivo	Self-inactivating (SIN) Gammaretroviral Vector	Autologous	Inflammatory and immune system	X-linked Severe Combined Immunodeficiency	Prof Adrian Thrasher UCL ICH
Genethon	A Phase I/II, Non Randomized, Multicenter, Open-label Study of g1xcgd (Lentiviral Vector Transduced CD34+ Cells) in Patients With X-linked Chronic Granulomatous Disease	X-linked chronic granulomatous disease (X-CGD) is a rare genetic disorder, which affects boys. The goal of this trial is to evaluate the safety and efficacy of transplantation of autologous CD34+ cells transduced with lentiviral vector containing XCGD gene in X-CGD patients.	NCT01855685	Great Ormond Street Hospital NHS Foundation Trust, London, UK UCL Institute of Child Health, London UK	University College London Hospital (UCLH) Recruiting London, United Kingdom, NW1 2PG Royal Free Hospital (RFH) Recruiting London, United Kingdom, NW3 2QG Great Ormond Street Hospital NHS Foundation Trust London, United Kingdom	3- Recruiting	Phase I/II	2013	5	CD34 and/or CD133 stem cells	Bone marrow	Yes ex-vivo	Lentiviral vector	Autologous	Inflammatory and immune system	X-Linked Chronic Granulomatous Disease (X-CGD)	Prof Adrian Thrasher UCL ICH
Bellicum Pharmaceuticals, USA	Phase I Study of CaspaCIDe T Cells From an HLA-partially Matched Family Donor After Negative Selection of TCR Alpha Beta T Cells in Pediatric Patients Affected by Hematological Disorders	This study will evaluate pediatric patients with malignant or non-malignant blood cell disorders who are having a blood stem cell transplant depleted of T cell receptor (TCR) alfa and beta cells that comes from a partially matched family donor. The study will assess whether T cells, from the family donor, that are specially grown in the laboratory and given back to the patient along with the stem cell transplant can help the immune system recover faster after transplant. As a safety measure these T cells have been programmed with a self-	NCT02065869	Bellicum Pharmaceuticals, USA	Institute of Child Health & Great Ormond Street Hospital, London The Newcastle Upon Tyne Hospitals NHS Foundation Trust, Newcastle	3- Recruiting	Phase I	2014	180	T cells	Blood	Yes ex-vivo	Retroviral vector expressing suicide gene iCasp9	Allogeneic	Blood	Hematological malignancies	Kirsty Devine Paediatric Research Nurse Great North Childrens Hospital Ward 1B, Research Unit Queen Victoria Road
Tetec AG, Germany	A Prospective Randomized Controlled Multicenter Phase-III Clinical Study to Evaluate the Safety and Effectiveness of NOVOCART® 3D Plus Compared to the Standard Procedure Microfracture in the Treatment of Articular Cartilage Defects of the Knee	Safety and Effectiveness Study to Evaluate NOVOCART® 3D Plus Compared to the Microfracture to Treat Articular Cartilage Defects of the Knee (N3D)	2011-005798-22 / NCT01656902	Tetec AG, Germany	Royal Devon and Exeter Hospital Exeter, United Kingdom, EX2 5DW	3- Recruiting	Phase III	2012	261	Chondrocytes	Tissue	No		Autologous	Bone and cartilage	Articular cartilage defects of the knee	Thomas Gwinner Alexandra Kirner

Cell and Gene Therapy Catapult UK Clinical Trials Database 2017

Name of Sponsor	Title	Project Summary	Clinical Database Numbers	Lead Institution/ Company and Collaborator Partners	United Kingdom Site(s)	Clinical Trial Status	Trial Phase	Year Trial Started	Recruitment Target	Cell Type	Cell Source	Gene Modification/ Gene	If applicable, type of virus vector used	Autologous/ Allogeneic	Disease Area	Indication	Contact
Institut de Recherches Internationales Servier, France	A phase 1, open label, non-comparative, monocenter study to evaluate the safety and the ability of UCART19 to induce molecular remission in paediatric patients with relapsed /refractory B acute lymphoblastic leukaemia (UCART19_PALL)	This study aims at evaluating the safety and efficacy of UCART19, an allogeneic CAR T-cell product for treatment of CD19-expressing hematological malignancies, gene edited with TALEN®, to induced molecular remission in pediatric patients with relapsed or refractory CD19-positive B-cell acute lymphoblastic leukemia (B-ALL) ahead of planned allogeneic hematopoietic stem cell transplantation (allo-HSCT).	NCT02808442	Institut de Recherches Internationales Servier, France	UCL Great Ormond Hospital, London, United Kingdom	3- Recruiting	Phase I	2016	10	T cells	Bone marrow	Yes ex-vivo	TALEN® gene editied cells	Autologous	Cancer (Haematology)	B-cell acute lymphoblastic leukemia	Institut de Recherches Internationales Servier clinicaltrials@servier.com
St Georges University London	Clinical development of erythrocyte encapsulated thymidine phosphorylase - a therapy for mitochondrial neurogastrointestinal encephalomyopathy	The aim of this trial is to evaluate erythrocyte encapsulated thymidine phosphorylase (EE-TP) in patients with mitochondrial neurogastrointestinal encephalomyopathy (MNGIE). Conducting a multi-centre (pan European), open-label, multiple ascending dose, Phase II trial in 10 patients with MNGIE, over 36 months		Orphan Technologies	N/A	1- In planning	Phase II	2016	10	Other	Other	No		Autologous	Metabolic and Endocrine	Mitochondrial neurogastrointestinal encephalomyopathy (MNGIE)	Bridget Bax bebax@sgul.ac.uk
Pfizer, UK	Phase 1, Open-label, Safety And Feasibility Study Of Implantation Of Pf-05206388 (Human Embryonic Stem Cell Derived Retinal Pigment Epithelium Living Tissue Equivalent) In Subjects With Acute Wet Age Related Macular Degeneration and Recent Rapid Vision Decline	A Study Of Implantation Of Retinal Pigment Epithelium In Subjects With Acute Wet Age Related Macular Degeneration	NCT01691261	University College, London	Moorfields Eye Hospital NHS Foundation Trust, London	4- Suspended	Phase I	2015	10	Retinal	Human embryonic stem cell	No		Allogeneic	Eye	Acute Wet Age Related Macular Degeneration	Peter T Loudon, Pfizer
Astellas Institute for Regenerative Medicine	Follow-up to 5 Years of a Phase I/II, Open-Label, Multi-Center, Prospective Study to Determine the Safety and Tolerability of Sub-retinal Transplantation of Human Embryonic Stem Cell Derived Retinal Pigmented Epithelial (hESC-RPE) Cells in Patients With Stargardt's Macular Dystrophy (SMD)	The purpose of this study is to evaluate the safety and tolerability of hESC-RPE cellular therapy in patients with advanced SMD over a five-year period following the surgical procedure to implant the cells.This study is a long-term, extension of a Phase I/II, open-label, non-randomized, 4-cohort, multi-center clinical trial (referred to as the core trial or core protocol) in which a maximum of 12 SMD patients were transplanted with sequential doses of hESC-RPE cells, starting at a dose of 50,000 hESC-RPE cells transplanted and increasing to a maximum dose of 200,000 hESC-RPE cells transplanted.	NCT02941991	Astellas Institute for Regenerative Medicine	Moorefields Eye Hospital NHS Foundation Trust, London, United Kingdom, EC1V2PD Newcastle on Tyne NHS Foundation Trust Newcastle upon Tyne, United Kingdom, NE7 7DN	5- In follow-up	Phase I/II	2013	11	Retinal	Human embryonic stem cell	No		Allogeneic	Eye	Stargardt's Macular Dystrophy	medinfo.gb@astellas.com
Cynata Therapeutics Limited	An Open-Label Phase 1 Study to Investigate the Safety and Efficacy of CYP-001 for the Treatment of Adults With Steroid-Resistant Acute Graft Versus Host Disease	The purpose of this study is to assess the safety, tolerability and efficacy of two infusions of CYP-001 in adults with steroid-resistant GvHD. This is a multi-centre, open label, dose escalation study to assess the safety, tolerability and efficacy of two infusions of CYP-001, in adults who have steroid-resistant GvHD. Participants will receive standard of care treatment throughout the study, according to local procedures. The first eight participants will be enrolled in Cohort A and receive a CYP-001 dose of 1 million cells per kg, up to a maximum dose of 100 million cells, on Day 0 and Day 7. Subject to a safety review of data from Cohort A, an additional eight participants will be enrolled into Cohort B and receive a CYP-001 dose of 2 million cells/kg, up to a maximum dose of 200 million cells, on Day 0 and Day 7. The primary evaluation period concludes for each participant 100 days after the first dose of CYP-001. Participants will have study visits on Days 0, 3, 7, 14, 21, 28, 60 and	NCT02923375	Cynata Therapeutics Limited	NHS Foundation Trust Recruiting Manchester, United Kingdom	3- Recruiting	Phase I	2016	16	Mesenchymal stem/stromal cells	Induced pluripotent stem cell	Yes ex-vivo		Allogeneic	Inflammatory and immune system	Steroid-Resistant Acute Graft Versus Host Disease	Jennifer Jardine
Belfast Health and Social Care Trust	Repair of Acute Respiratory Distress Syndrome by Stromal Cell Administration (REALIST): An Open Label Dose Escalation Phase 1 Trial Followed by a Randomized, Double-blind, Placebo-controlled Phase 2 Trial	Acute Respiratory Distress Syndrome (ARDS) causes the lungs to fail due to the collection of fluid in the lungs (pulmonary oedema). ARDS is common in severely ill patients in Intensive Care Units and is associated with a high mortality and a high morbidity in those who survive. There is a large economic burden with direct healthcare costs, but also indirectly due to the impact on the carer and patient through the patients inability to return to full time employment. There is little evidence for effective drug (pharmacological) treatment for ARDS. There is increasing information that mesenchymal stem cells (MSCs) might be important in treating ARDS. REALIST will investigate if a single infusion of MSCs will help in the treatment of ARDS. The first step will be to first of all determine what dose of MSCs is safe and then divide patients suffering from ARDS into two groups, one of which will get MSCs and the other a harmless dummy (or placebo) infusion, who will then be followed up to	NCT03042143 Eudract 2017-000584-33	Belfast Health and Social Care Trust Queen's University, Belfast Northern Ireland Clinical Trials Unit	Belfast Health and Social Care Trust, Royal Hospitals	2- In set-up	Phase I/II	2017	84	Mesenchymal stem/stromal cells	Other	No		Allogeneic	Other	Acute Respiratory Distress Syndrome	Danny F McAuley, MD Cecilia O'Kane, Ph.D
Bluebird Bio	A Phase 3 Single Arm Study Evaluating the Efficacy and Safety of Gene Therapy in Subjects With Transfusion-dependent β-Thalassemia, Who do Not Have β0/β0 Genotype, by Transplantation of Autologous CD34+ Stem Cells Transduced Ex Vivo With a Lentiviral βA-T87Q-Globin Vector in Subjects ≥12 and ≤50 Years of Age	This is a single-arm, multi-site, single-dose, Phase 3 study in approximately 15 subjects ≥12 and ≤50 years of age with transfusion-dependent β-thalassemia (TDT), also known as β-thalassemia major, who do not have a β0 mutation at both alleles of the hemoglobin β (HBB) gene. The study will evaluate the efficacy and safety of autologous hematopoietic stem cell transplantation (HSCT) using LentiGlobin BB305 Drug Product.	NCT02906202	Bluebird Bio	London	2- In set-up	Phase III	2016	15	CD34 and/or CD133 stem cells	Bone marrow	Yes ex-vivo	Lentiviral βA-T87Q-Globin Vector	Autologous	Blood	Transfusion-Dependent β-Thalassemia	clinicaltrials@bluebirdbio.com

Name of Sponsor	Title	Project Summary	Clinical Database Numbers	Lead Institution/ Company and Collaborator Partners	United Kingdom Site(s)	Clinical Trial Status	Trial Phase	Year Trial Started	Recruitment Target	Cell Type	Cell Source	Gene Modification/ Gene	If applicable, type of virus vector used	Autologous/ Allogeneic	Disease Area	Indication	Contact
Servier	Phase I, Open Label, Dose-escalation Study to Evaluate the Safety, Expansion, Persistence and Biological Activity of a Single Dose of UCART19 (Allogeneic Engineered T-cells Expressing Anti-CD19 Chimeric Antigen Receptor), Administered Intravenously in Patients With Relapsed or Refractory CD19 Positive B-cell Acute Lymphoblastic Leukaemia (B-ALL) or Chronic Lymphocytic Leukaemia (CLL)	The purpose of this study is to evaluate the safety and tolerability of several doses of UCART19 in patient with relapsed / refractory (R/R) acute lymphoblastic leukaemia (ALL) or chronic lymphocytic leukaemia (CLL)	NCT02746952	Servier	King's College Hospital NHS Foundation Trust	3- Recruiting	Phase I	2016	12	T cells		Yes ex-vivo		Allogeneic	Cancer (Haematology)	Acute Lymphoblastic Leukaemia (ALL) and Chronic Lymphocytic Leukaemia (CLL) (CALM)	Institut de Recherches Internationales Servier
Servier	A Phase 1, Open Label, Non-comparative, Monocenter Study to Evaluate the Safety and the Ability of UCART19 to Induce Molecular Remission in Paediatric Patients With Relapsed/Refractory B Acute Lymphoblastic Leukaemia	This study aims at evaluating the safety and ability of UCART19 to induce molecular remission in pediatric patients with relapsed or refractory CD19-positive B-cell acute lymphoblastic leukemia (B-ALL) ahead of planned allogeneic hematopoietic stem cell transplantation (allo-HSCT).	NCT02808442	Servier	UCL Great Ormond Hospital	3- Recruiting	Phase I	2016	10	T cells		Yes ex-vivo		Allogeneic	Cancer (Haematology)	Relapsed/Refractory B Acute Lymphoblastic Leukemia (PALL)	Institut de Recherches Internationales Servier
University of Oxford	Gene Therapy for Blindness Caused by Choroideremia	An Open Label Dose Escalation Phase 1 Clinical Trial of Retinal Gene Therapy for Choroideraemia Using an Adeno-associated Viral Vector (AAV2) Encoding Rab-escort Protein 1 (REP1)	NCT01461213	Collaborators: Oxford University Hospitals NHS Trust Moorfields Eye Hospital NHS Foundation Trust University College, London Central Manchester University Hospitals NHS Foundation Trust	Moorfields Eye Hospital NHS Foundation Trust St Mary's Hospital, Central Manchester University Hospitals NHS Foundation Trusts Oxford Radcliffe Hospitals NHS Trust	5- In follow-up	Phase I	2011	14			Yes in-vivo	rAAV2		Eye	Choroideraemia	Robert E MacLaren
Oxford BioMedica	A Multicentre, Open-label Study to Determine the Long Term Safety, Tolerability and Efficacy of ProSavin in Patients With Bilateral, Idiopathic Parkinson's Disease.	This study is designed to determine the long term (10 years) safety, tolerability and efficacy of ProSavin, a lentiviral based vector carrying three genes that encode the key enzymes for the synthesis of dopamine, in patients with bilateral, idiopathic Parkinson's disease who received the ProSavin in previous study (PS1/001/07).	NCT01856439	Henri Mondor Hospital Paris, France Addenbrookes Hospital Cambridge	Addenbrookes Hospital Cambridge	5- In follow-up	Phase I/II	2011	15			Yes in-vivo	Lentiviral vector		Neurological	Parkinson's Disease	Oxford BioMedica
GenSight Biologics	A Randomized, double-masked, sham-controlled clinical trial to evaluate the efficacy of a single intravitreal injection of GS010 in subjects affected for 6 months or less by Leber Hereditary Optic Neuropathy (LHON) due to the G11778A mutation in the	The goal of this study is to assess the efficacy of GS010, a gene therpy, in improving the visual outcome in patients up to 6 months from onset of Leber Hereditary Optic Neuropathy (LHON) due to the ND4 mitochondrial mutation (RESCUE)	NCT02652767	GenSight Biologics, France	Moorfields Eye Hospital NHS Foundation Trust, London	3- Recruiting	Phase III	2016	36			Yes in-vivo	GS010: recombinant adeno-associated viral vector serotype 2 (rAAV2/2) containing the wild-type ND4		Eye	Leber Hereditary Optic Neuropathy (LHON)	Lauren Leitch-Devlin Moorfields Eye Hospital NHS Foundation Trust
GenSight Biologics	Randomized, Double-Masked, Sham-Controlled Clinical Trial to Evaluate the Efficacy of a Single Intravitreal Injection of GS010 in Subjects Affected for More Than 6 Months and To 12 Months by LHON Due to the G11778A Mutation in the ND4 Gene	The goal of this study is to assess the efficacy of GS010, a gene therpy, in improving the visual outcome in patients with LHON due to the G11778A ND4 mitochondrial mutation when vision loss is present for more than six months and up to one year (REVERSE)	NCT02652780	GenSight Biologics, France	Moorfields Eye Hospital NHS Foundation Trust, London	5- In follow-up	Phase III	2016	36			Yes in-vivo	GS010: recombinant adeno-associated viral vector serotype 2 (rAAV2/2) containing the wild-type ND4		Eye	Leber Hereditary Optic Neuropathy (LHON)	Lauren Leitch-Devlin Moorfields Eye Hospital NHS Foundation Trust
BioMarin Pharmaceutical	Gene Therapy Study in Severe Haemophilia A Patients	A Phase 1/2, Dose-Escalation Safety, Tolerability and Efficacy Study of BMN 270, an Adenovirus-Associated Virus Vector-Mediated Gene Transfer of Human Factor VIII in Patients With Severe Haemophilia A	NCT02576795 EudraCT: 2014-003880-38	BioMarin Pharmaceutical	Hampshire Hospitals NHS Foundation Trust, Basingstoke Queen Elizabeth Hospital Birmingham University Hospitals Bristol NHS Foundation Cambridge University Hospitals NHS Foundation Greater Glasgow Health Board Barts Health NHS Trust, London	3- Recruiting	Phase I/II	2015	15			Yes in-vivo	AAV		Blood	Haemophilia A	BioMarin Pharmaceutical
Ionis Pharmaceuticals, Inc.	A Randomized, Double-blind, Placebo-controlled Study to Evaluate the Safety, Tolerability, Pharmacokinetics and Pharmacodynamics of Multiple Ascending Doses of Intrathecally Administered ISIS 443139 in Patients With Early Manifest Huntington's Disease	This study will test the safety, tolerability, pharmacokinetics and pharmacodynamics of multiple ascending doses of IONIS-HTTRx administered intrathecally to adult patients with early manifest Huntington's Disease.	NCT02519036	Ionis Pharmaceuticals, Inc.	University Hospitals Birmingham Cambridge University Hospital University College London Hospitals University Hospital Wales University of Manchester, St. Mary's Hospital	3- Recruiting	Phase I/II	2015	44			Yes in-vivo	Single stranded antisense oligonucleotide (ASO)		Neurological	Huntington's disease	patients@ionisph.com

Name of Sponsor	Title	Project Summary	Clinical Database Numbers	Lead Institution/ Company and Collaborator Partners	United Kingdom Site(s)	Clinical Trial Status	Trial Phase	Year Trial Started	Recruitment Target	Cell Type	Cell Source	Gene Modification/ Gene	If applicable, type of virus vector used	Autologous/ Allogeneic	Disease Area	Indication	Contact
MeiraGTx UK II Ltd	An Open Label, Multi-centre, Phase I/II Dose Escalation Trial of a Recombinant Adeno-associated Virus Vector (AAV2/8-hCARp.hCNGB3) for Gene Therapy of Adults and Children With Achromatopsia Owing to Defects in CNGB3	Achromatopsia is a recessively inherited condition characterised by a lack of cone photoreceptor function resulting in impairment of colour vision and visual acuity, central scotoma often with eccentric fixation, disabling hypersensitivity to light (photophobia) and involuntary eye movements (pendular nystagmus). Children with CNGB3-related achromatopsia have profound sight impairment from birth or early infancy. The condition is currently untreatable, but there is a real possibility that a gene therapy could offer a significant benefit in terms of improved sight and quality of life (QOL), based on our own and others experience of ocular gene therapy trials and pre-clinical data demonstrating	NCT03001310	EMAS Syne Qua Non Limited	Moorfields Eye Hospital NHS Foundation Trust, London, UK	3- Recruiting	Phase I/II	2016	18			Yes in-vivo	AAV2/8 viral vector		Eye	Achromatopsia	Julie Bakobaki, MSc Anna Morka, MSc
University College London	An Open-label, Multi-centre, Phase I/II Dose Escalation Trial of an Adeno Associated Of an Adeno-Associated Virus Vector (AAV2/5-OPTIRPE65) for Gene Therapy of Adults And Children With Retinal Dystrophy Associated With Defects in RPE65 (LCA)	The inherited retinal dystrophies are a clinically and genetically heterogeneous group of conditions, which often present in childhood.Inherited retinal degenerations cause sight impairment in approximately 1 in 3000 people in the Western world. There are currently no effective treatments. Leber congenital amaurosis (LCA) is a severe, early-onset form of inherited retinal degeneration involving both rod and cone photoreceptors. LCA is caused by mutations in one of at least 19 different genes.Mutations in RPE65, which is expressed in the retinal pigment epithelium (RPE), are responsible in 3 to 16 % of people affected. The RPE65 gene encodes a 65-kDa retinal pigment epithelium (RPE)-specific protein that is required for the conversion of vitamin A to 11-cis-retinal by the RPE and is essential for the regeneration of the rod visual pigment. Proof of principle studies for RPE65 gene replacement therapy has been demonstrated with the use of recombinant adeno-associated virus serotype 2	NCT02781480	Medical Research Council MeiraGTx UK II Ltd	Moorfields Eye Hospital NHS Foundation Trust Recruiting London, United Kingdom, EC1V 2PD	3- Recruiting	Phase I/II	2016	27			Yes in-vivo	AAV2/5-OPTIRPE65		Eye	Leber Congenital Amaurosis	James Bainbridge
MeiraGTx UK II Ltd	Long-term Follow-up Study of Participants Following an Open Label, Multi-centre, Phase I/II Dose Escalation Trial of an Adeno-associated Virus Vector (AAV2/5-OPTIRPE65) for Gene Therapy of Adults and Children With Retinal Dystrophy Owing to Defects in RPE65 (LCA2)	This study is a longer-term follow-up study for patients who have been administered AAV2/5-OPTIRPE65 in the Phase I/II, open label, non-randomised, two-centre, dose escalation trial in adults and children with retinal dystrophy associated with defects in RPE65. The study is designed to collect data on longer-term safety and efficacy at 9-, 12-, 18-, 24-, 36-, 48- and 60-month time-points following AAV2/5-OPTIRPE65 administration.	NCT02946879	Syne Qua Non Limited	Moorfields Eye Hospital NHS Foundation Trust Recruiting London, United Kingdom	3- Recruiting	Phase I/II	2016	27			Yes in-vivo	AAV2/5-OPTIRPE65		Eye	Leber Congenital Amaurosis	Sophie Connor Neruban Kumaran, Dr
University College, London	GO-8: Gene Therapy for Haemophilia A Using a Novel Serotype 8 Capsid Pseudotyped Adeno-associated Viral Vector Encoding Factor VIII-V3	Haemophilia A is an x-linked, life threatening bleeding disorder arising from defects in the coagulation factor VIII (FVIII) gene. Current treatment for haemophilia A, the commonest inherited bleeding disorder (prevalence of 1 in 5000 individuals) consists of life-long, 2-3X/week, intravenous injection of clotting factor concentrates, which is demanding, exceedingly expensive not widely available nor curative. In contrast, gene therapy offers the potential of a cure for haemophilia A as illustrated by our first unequivocal success in a related condition, haemophilia B. In that study the investigators showed that a single intravenous administration of a serotype 8 based adeno-associated virus, (AAV8) vector encoding the factor IX (FIX) gene resulted in stable (>6 years) therapeutic expression of FIX without long-lasting toxicity. The investigators plan to use the same AAV8 platform to evaluate a novel FVIII expression cassette, AAV2/8-HLP-FVIII-V3, in patient with haemophilia A. Extensive	NCT03001830	Medical Research Council	Royal Free Hospital London, United Kingdom, NW3 2QG Principal Investigator: Pratima Chowdary Sub-Investigator: Amit Nathwani Sub-Investigator: Edward Tuddenham	2- In set-up	Phase I	2016	18			Yes in-vivo	AAV2/8-HLP-FVIII-V3		Blood	Haemophilia A	Thomas Roberts