Cell and Gene Therapy Catapult UK clinical trials database 2016

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 therapy	If applicable, type of virus vector used	Autologous/ allogeneic	Disease area	Clincial indication	Contact
Cell Medica Ltd., UK	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.	EudraCT: 2011-001788-36	Cell Medica	Great Ormond Street Hospital London, Royal Manchester Children's Hospital, Royal Victoria Infirmary	Recruiting	Phase I/II	2012	15 treated patients	T cells	Blood	No		Allogeneic	Cancer (haematology)	ADV in paediatric patients following bone marrow transplantation	Karen Hodgkin, Cell Medica (karen.hodgkin@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).	EudraCT: 2006-004950-25 NCT01621724	University College London		Recruiting	Phase I/II	2012	18	T cells	Blood	Yes *ex-vivo*	Gamma-retrovirus	Autologous	Cancer (haematology)	Acute myeloid leukaemia; chronic myeloid leukaemia	Paloma Salazar Senior CMP Cell and Gene Therapy Catapult paloma.salazar@ct.catapult.org.uk
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.	EudraCT: 2007-000684-16	Great Ormond Street Hospital, London	Great Ormond Street Hospital, London	Recruiting	Phase I/II	2011	10	CD34 and/or CD133 stem cells	Blood and bone marrow	Yes *ex-vivo*	Self-inactivating (SIN) Gammaretrovirus	Autologous	Blood	X-linked severe combined immunodeficiency	Havinder Hara or Cecile Duret Clinical Project Manager UCL Institute of Child Health London h.hara@ucl.ac.uk or c.duret@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 autologous 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	EudraCT: 2010-024253-36 NCT01380990	Great Ormond Street Hospital, London	Great Ormond Street Hospital, London	In follow-up	Phase I/II	2012	10	CD34 and/or CD133 stem cells	Blood and bone marrow	Yes *ex-vivo*	Lentiviral vector	Autologous	Blood	Adenosine deaminase deficiency	Havinder Hara or Cecile Duret Clinical Project Manager UCL Institute of Child Health London h.hara@ucl.ac.uk or c.duret@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 Hospital, UCLH Foundation Trust, London The Royal Free Hospital, Royal Free London Foundation Trust, London	In follow-up	Phase I/II	2007	70	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	In follow-up	Phase II	2011	180 (3000)	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)
Imperial College London	Stem cells in rapidly evolving active multiple sclerosis	Stem cells in rapidly evolving active multiple sclerosis (STREAMS).	UK CRN 13496 NCT01606215 EudraCT: 2012-002357-35	Imperial College London	Imperial College Healthcare	In follow-up	Phase II	2012	13	Mesenchymal stem/stromal cells	Bone marrow	No		Autologous	Neurological	Relapsing remitting multiple sclerosis/ secondary progressive multiple sclerosis/ primary progressive multiple sclerosis	Anne Bradshaw, Imperial College Healthcare NHS Trust anne.bradshaw@imperial.nhs.uk d.wilkie@imperial.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	University of Cambridge Lund University Cardiff University Imperial College London University College London University Hospital Freiburg Life Science Governance Institute Assistance Publique - Hopitaux de Paris Institut National de la Santé Et de la Recherché Medicale, France Life Technologies Ltd Inomed Cambridge Cognition Ltd Skane University Hospital Imanova Limited	Cardiff University Imperial College London University College London University of Cambridge	In follow-up	Phase I/II	2012	40: 20 transplanted patients, 20 controls	Neural	Fetal ventral mesencephalic tissue	No		Allogeneic	Neurological	Parkinson's disease	Natalie Valle Guzman Transeuro Trial Manager University of Cambridge
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 NCT01151124	Glasgow Southern General Hospital	Glasgow Southern General Hospital	In follow-up	Phase I	2010	11	Neural	Brain tissue	No		Allogeneic	Neurological	Stroke disability	Dr John Sinden ReNeuron Ltd. info@reneuron.com

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ReNeuron Limited, UK	A Phase II Simon Optimal Two Stage 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 NCT02117635	Glasgow Southern General Hospital	Queen Elizabeth Hospital, Birmingham NHS Southern General Hospital, Glasgow King's College Hospital, London University College London Hospital Royal Victoria Infirmary, Newcastle Nottingham City Hospital Salford Royal NHS Foundation Trust Royal Hallamshire Hospital, Sheffield Southampton Hospital	Recruiting	Phase II	2014	41	Neural	Brain 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 NCT01916369	Ninewells Hospital, Dundee	Ninewells Hospital, Dundee	Recruiting	Phase I	2014	9	Neural	Brain 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 (EBMT)	Autologous stem cell transplantation international Crohn's disease trial	Autologous CD34+ haematopoietic cells for Crohn's disease.	EudraCT: 2005-003337-40 ISRT39133198 UK CRN 7107	European Group for Blood and Marrow Transplantation (EBMT)	Nottingham University Hospital	In follow-up	Phase II/III	2006	45	CD34 and/or CD133 stem cells	Bone marrow	No		Autologous	Oral and gastrointestinal	Crohn's disease	Prof Hawkey, NDDC University Hospital, QMC, Nottingham NG7 2UH cj.hawkey@nottingham.ac.uk Trial Coordinator: Miranda Clark astic@nottingham.ac.uk
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).	EudraCT: 2011-000608-16 ISRCTN51772481 UK CRN 11185	Newcastle University		In follow-up	Phase II	2012	24	Corneal	Limbus	No		Autologous	Eye	Limbal stem cell deficiency	Professor Francisco C Figueiredo, Newcastle University, UK
Ocata Therapeutics, USA	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)	Retinal pigment epithelial cell replacement for Stargardt's disease.	NCT01469832	Ocata Therapeutics		In follow-up	Phase I/II	2011	12	Retinal	Human embryonic stem cell	No		Allogeneic	Eye	Stargardt's disease	Dr James Bainbridge Moorfields Eye Hospital j.bainbridge@ucl.ac.uk
Dompé Farmaceutici S.p.A	A Phase 3, Multicenter, Randomized, Double-blind, Parallel Assignment Study to Assess the Efficacy and Safety of Reparixin in Pancreatic Islet Transplantation	A Phase 3, multicenter, randomized, double-blind, parallel assignment study to assess the efficacy and safety of Reparixin in pancreatic islet transplantation.	NCT01817959	Dompé Farmaceutici S.p.A	Institute of Transplantation, Newcastle upon Tyne Hospitals	In follow-up	Phase III	2012	42	Pancreatic islets		No			Diabetes	Diabetes Mellitus Type 1	Prof James Shaw Institute of Cellular Medicine Newcastle University
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		Recruiting	Phase III	2007	100	Pancreatic islets	Deceased donor pancreas	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 expanded stem 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.	EudraCT: 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	Recruiting	Phase II	2013	114	Mesenchymal stem/stromal cells and Chondrocytes	Bone marrow/cartilage	No		Autologous and allogeneic	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.	EudraCT: 2010-024162-22	Azellon Cell Therapeutics		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 University 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.	NCT01352858 ISRCTN87426082 UK CRN 12108	Newcastle University		Recruiting	Phase I	2011	12	Antigen presenting cells	Blood	No		Autologous	Musculoskeletal	Rheumatoid and inflammatory arthritis	Prof John Isaacs Newcastle University Institute of Cellular Medicine j.d.isaacs@ncl.ac.uk

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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	Recruiting	Phase II	2014	24	T cells	Blood	No		Allogeneic	Cancer (haematology)	Haematological malignancies	ICAT trial coordinator Cancer Research UK & UCL Cancer Trials Centre London ctc.icat@ucl.ac.uk
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	Recruiting	Phase I	2015	21	T cells	Blood	Yes *ex-vivo*	Retroviral vector	Autologous	Cancer	Locally advanced/ recurrent head and neck cancer for which no suitable alternative therapy is available	John Maher King'sCollege London, john.maher@kcl.ac.uk
Dendreon Corporation, USA	An open-label study of sipuleucel-T in European men with metastatic, castrate resistant prostate cancer	An open-label study of sipuleucel-T in European men with metastatic, castrate resistant prostate cancer	EudraCT: 2011-001192-39	Barts London Hospital		Recruiting	Phase II	2012	45	Antigen presenting cells	Blood	No		Autologous	Cancer	Metastatic, castrate resistant prostate cancer	Abi Foreshew, ECMC, Barts Cancer Institute (Clinical Trials Practitioner)
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		In set-up	Phase I	2014	60	Neural	Brain tissue	No		Allogeneic	Neurological	Neurological	Prof Stephen Dunnett The Brain Repair Group, School of Biosciences Cardiff University, 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		Recruiting	Phase I/II	2014	26	T cells	Blood	No		Autologous	Oral and gastrointestinal	End-stage liver disease	Alberto Sanchez-Fueyo, MD, PhD Gavin Whitehouse, BM, MRCP(UK)
North Bristol NHS Trust	Repeat Infusion of Autologous Bone Marrow Cells in Multiple Sclerosis (SIAMMS-II)	The purpose of this study is to test the safety of repeated bone marrow stem cell infusion in patients with MS. We want to find out what effects, good and/or bad, it has on you and your disability. The results of a previous safety study of bone marrow stem cell infusion in patients with MS raised the possibility of some early partial repair; measurements of the speed of neurological impulses in the brain and spinal cord improved. The current study seeks to determine whether those benefits have persisted and whether they can be repeated or enhanced by repeating the procedure.	NCT01932593	Sir Halley Stewart Trust		Recruiting	Phase I	2014	6	Bone marrow mononuclear cells	Bone marrow	No		Autologous	Neurological	Multiple sclerosis	claire.rice@nbt.nhs.uk; heather.williams@nbt.nhs.uk
North Bristol NHS Trust	Assessment of Bone Marrow-derived Cellular Therapy in Progressive Multiple Sclerosis (ACTiMuS)	We have previously performed a preliminary safety study of bone marrow stem cell infusion in a small number of patients with MS. The results raised the possibility of some early partial repair; measurements of the speed of neurological impulses in the brain and spinal cord improved. The current trial is a more comprehensive study to examine whether this was a true result and help us to understand the mechanisms involved so that we can further improve therapy for MS.	NCT01815632 ISRCTN27232902	Silverman Family Foundation, Medical Research Council		Recruiting	Phase II	2014	80	Bone marrow mononuclear cells	Bone marrow	No		Autologous	Neurological	Multiple sclerosis	claire.rice@nbt.nhs.uk; heather.williams@nbt.nhs.uk
University College London	Clinical Trial of Stem Cell Based Tissue Engineered Laryngeal Implants (RegenVOX)	This study aims to test a new treatment for narrowing of the voicebox and upper windpipe, which can be due to injury, inflammatory disease or cancer treatment. The treatment is an implant based on a human donor voicebox or windpipe that has been processed in order to remove all the cells from the donor. The patient's own stem cells are removed from the bone marrow, then are grown on the scaffold in the laboratory. A split skin graft from the patient may be needed to line the inside of the implant. Once the scaffold is ready to be implanted into the patient, an operation is performed, which final stage involves removing the narrow section of voicebox or upper windpipe and implanting the scaffold to reconstruct it. Patients will be followed up for two years, with investigations such as CT scans, examination of the voicebox and windpipe with a flexible camera (bronchoscopy) and blood tests performed at specific times.	NCT01977911	University College, London		Recruiting	Phase I/II	2015	10	Bone marrow mononuclear cells	Bone marrow	No		Autologous	Respiratory	Ear, nose and throat	Prof Martin Birchall
Cell Medica Inc, UK	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	NCT01948180	Cell Medica/ 24 clinical sites, US, UK, Fr, De and SK	University College London Hospital, London The Christie Clinic, Manchester	Recruiting	Phase II	2015	35	T cells	Blood	No		Autologous	Cancer (haematology)	NK/T cell lymp[homa	Karen Hodgkin, Cell Medica karen.hodgkin@cellmedica.co.uk

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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		In follow-up	Phase I/II	2014	12	T cells	Blood	No		Autologous	Renal and urogenital	End-stage kidney disease	Dr Rachel Hilton BMBCh PhD
Cell and Gene Therapy Catapult Ltd	A Phase I/II study of the safety and efficacy of gene-modified WT1 TCR therapy in patients with Myelodysplastic Syndrome (MDS) and Acute Myeloid Leukaemia (AML)	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) with low blast counts failing to achieve an IWG defined response following azacitidine therapy.	EudraCT: 2014-003111-10	University College Hospital London		Recruiting	Phase I/II	2015	25	T cells	Blood	Yes *ex-vivo*	Gamma-retrovirus	Autologous	Cancer (haematology)	Myeloidysplastic syndrome and acute myeloid leukaemia	Paloma Salazar Senior CMP Cell and Gene Therapy Catapult paloma.salazar@ct.catapult.org.uk
Cell and Gene Therapy Catapult Ltd	Decellularised cadaveric tracheal scaffold recellularised with autologousmesenchymal stromal cells (MSCs)	This is a phase I study to evaluate the safety, efficacy and tolerability 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	EudraCT: 2015-002108-10	University College London Videregen		In set-up	Phase I	Expected 2015	4	Mesenchymal stem/stromal cells	Bone marrow	No		Autologous	Respiratory	Tracheal stenosis and tracheomalacia	Prof Martin Birchall University College London Gareth Wright Cell Therapy Catapult
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	EudraCT: 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	Recruiting	Phase I/II	Expected 2015	40	MultiStem®: multipotent adult progenitor cells manufactured from adult bone marrow	Bone marrow	No		Allogeneic	Respiratory	Acute respiratory distress syndrome	Paloma Salazar Cell 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	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 London 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 implantation.	EudraCT: 2010-021463-32	ICTA company (CRO)/University College London Hospitals		In follow-up	Phase II	2013	252	Skeletal muscle cells	Muscle-derived tissue	No		Autologous	Oral and gastrointestinal	Faecal incontinence	Susanne Hörl Clinical Project Manager 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		Recruiting	Phase I/II	2012	50	Skeletal Muscle Cells	Muscle-derived tissue	No		Autologous	Musculoskeletal	Faecal incontinence	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	Royal National Orthopaedic Hospital		Recruiting	Phase II	2015	10	Mesenchymal stem/stromal cells	Other	No		Autologous	Musculoskeletal	Achilles tendinopathy	Andrew Golberg Royal National Orthopaedic Hospital andy.goldberg@rnoh.nhs.uk
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		In set-up	Phase I	2015	12	T cells	Blood	Yes *ex-vivo*	Lentiviral vector	Autologous	Cancer (Haematology)	Diffuse large B-cell lymphoma	COBALT trial coordinator ctc.cobalt@ucl.ac.uk

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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.	EudraCT: 2015-001144-10	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, London University College Hospital, London Manchester Royal Infirmary, Manchester	Recruiting	Phase I	2015	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		Recruiting	Phase I/II	2015	37	Bone marrow mononuclear cells	Blood	No		Autologous	Liver		Prof Stuart Forbes University of Edinburgh Centre for Regenerative Medicine MRC, Edinburgh 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	Leiden University Medical Center, Leiden, The Netherlands A.O. Ospedale Papa Giovanni XXIII, Bergamo, Italy IRCCS - Istituto di Ricerche Farmacologiche Mario Negri, Bergamo, Italy National University of Ireland, Galway, Ireland	NHS Blood and Transplant, Liverpool University Hospital Birmingham, Birmingham, and Belfast Health and Social Care Trust, Belfast	In set-up	Phase I/II	2016	48	Mesenchymal stem/stromal cells	Bone marrow	No		Allogeneic	Diabetes	Diabetic kidney disease	Peter Maxwell, MD Belfast City Hospital Paul Cockwell, MD Queen Elizabeth Medial Centre
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		Hammersmith Hospital, London	Recruiting	Phase II	2015	15	ATIR101: a T-lymphocyte Enriched Leukocyte Preparation Depleted ex Vivo of Host Alloreactive T-cells (Using Photodynamic Treatment)	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		University of Edinburgh Leeds University & Leeds Teaching Hospitals NHS Trust Newcastle University	Recruiting	Phase I/II	2016	44	ProtheraCytes: autologous PB-CD34+ Stem Cells after automated ex-vivo expansion with the StemXpand machine	Bone marrow	No		Autologous	Cardiovascular	Acute myocardial infarction	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		Guy's and St Thomas NHS Trust, London Great Ormond Street Hospital for Children NHS Trust , London	Recruiting	Phase I	2014	5	Autologous epidermal sheets generated from genetically modified skin stem cells	Tissue	Yes *ex-vivo*	Lentiviral vector	Autologous	Skin	Netherton syndrome	Dr Waseem Qasim Anne-McNicol Dr Marie McNicol
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		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		Recruiting	Phase I/II	2011	10	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		Recruiting	Phase I/II	2013	20	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

Cell and Gene Therapy Catapult UK clinical trials database 2016

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 therapy	If applicable, type of virus vector used	Autologous/ allogeneic	Disease area	Clincial indication	Contact
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-destruct switch so that they can be destroyed if they start to react against tissues (Graft versus host disease).	NCT02065869		Institute of Child Health & Great Ormond Street Hospital, London The Newcastle Upon Tyne Hospitals NHS Foundation Trust, Newcastle	Recruiting	Phase I	2014	30	T cells	Blood	Yes *ex-vivo*	Retroviral vector expressing suicide gene iCasp9	Allogeneic	Cancer (haematology)	Hematological malignancies	Kirsty Devine Paediatric Research Nurse Great North Childrens Hospital Newcastle Upon Tyne Tel: 01912820607
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).	EudraCT: 2011-005798-22 NCT01656902		The Royal Orthopaedic Hospital, Birmingham Royal Devon and Exeter Hospital, Exeter	Recruiting	Phase III	2012	261	Chondrocytes	Osteochondral cylinders (derived from arthroscopy)	No		Autologous	Bone and cartilage	Articular cartilage defects of the knee	Thomas Gwinner Manja Meyer
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		UCL Great Ormond Hospital, London, United Kingdom	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		In planning	Phase II	2016	10	Erythrocytes	Blood	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 (Rpe) 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	In follow-up	Phase I	2015	10	Retinal cells	Human embryonic stem cell	No		Allogeneic	Eye	Acute wet age related macular degeneration	Peter T Loudon, Pfizer
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	Oxford University Hospitals NHS Trust Moorfields Eye Hospital NHS Foundation Trust University College, London Central Manchester University Hospitals NHS Foundation Trust University of Manchester University Hospital Southampton NHS Foundation Trust. University of Southampton	Oxford Eye Hospital	Recruiting	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	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 mitochondrial ND4 gene	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	Recruiting	Phase III	2016	36			Yes *in-vivo*	GS010: recombinant adeno-associated viral vector serotype 2 (rAAV2/2) containing the wild-type ND4 gene (rAAV2/2-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	Recruiting	Phase III	2016	36			Yes *in-vivo*	GS010: recombinant adeno-associated viral vector serotype 2 (rAAV2/2) containing the wild-type ND4 gene (rAAV2/2-ND4).		Eye	Leber Hereditary Optic Neuropathy (LHON)	Lauren Leitch-Devlin Moorfields Eye Hospital NHS Foundation Trust

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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		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 Guy's & St. Thomas' NHS Foundation Trust, London Imperial College Healthcare NHS Trust, London	Recruiting	Phase I/II	2015	12			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	Roche	Cambridge University Hospital University College London University of Manchester, St. Mary's Hospital	Recruiting	Phase I/II	2015	36			Yes *in-vivo*	Single stranded antisense oligonucleotide (ASO)		Neurological	Huntington's disease	patients@ionisph.com