

**VERIFIED CELL THERAPY CLINICAL TRIALS IN THE UK**

Project summary	Lead institution/ company	Present stage of development	Year current trial started	Recruitment target for current trial	Current trial still actively recruiting	Cell type	Autologous/ Allogeneic	Cell source	Disease area	Indication	Contact	I agree to this data being used in a publicly available database that shows clinical trial activity in the UK cell-based therapy industry
Cytovir CMV (cytomegalovirus) adoptive T cell therapy for CMV immunity post bone marrow transplantation from sibling donor (IMPACT study). T cells derived from sibling donor providing bone marrow	Cell Medica	Phase 3	2008	Minimum 70	Yes	T cell	Allogeneic	Peripheral blood stem cells	Oncology/ Blood	CMV reactivation following allogeneic haematopoietic stem cell transplantation (prophylactic)	Karen Hodgkin, Cell Medica (karen.hodgkin@cellmedica.co.uk)	Yes
Cytomegalovirus (CMV) vaccination using adoptive T cell transfer following haematopoietic stem cell transplantation (ACE/ASPECT study)	Cell Medica with Birmingham University	Phase 2	2010	42	Yes	T cell	Allogeneic	Peripheral blood stem cells	Oncology/ Blood	CMV reactivation following allogeneic haematopoietic stem cell transplantation (pre-emptive)	Karen Hodgkin, Cell Medica (karen.hodgkin@cellmedica.co.uk)	Yes
Adoptive T cell therapy for the reconstitution of immunity to adenovirus (ADV) in paediatric patients following bone marrow transplantation	Cell Medica	Phase 1/2	2012	15 treated patients	Yes	T cell	Allogeneic	Immune cells	Oncology/ Blood	ADV in paediatric patients following bone marrow transplantation	Karen Hodgkin, Cell Medica (karen.hodgkin@cellmedica.co.uk)	Yes
WT1 TCR gene therapy for leukaemia: a phase I/II safety and toxicity study (WT1 TCR-001)	University College London	Phase I/2	2012	18	Yes	Transduced T cell	Autologous	Peripheral Blood	Oncology/ Blood	Acute myeloid leukaemia; chronic myeloid leukaemia	Dr Emma Morris (CI), UCL e.morris@ucl.ac.uk	Yes
A Phase II trial to assess the activity of NY-ESO-1 targeted T cells in advanced oesophagogastric cancer. Gene modified T cells expressing an engineered TCR to recognise NY-ESO-1 cancer antigen	The Christie NHS Foundation Trust, Manchester, UK (Treatment centre and 6 other sites across EU); Cellular Therapeutics Ltd, UK - IMPD manufacturing	Phase 2. Target date for trial completion 2018				Engineered T cells	Autologous	Peripheral blood	Oncology	Advanced oesophagogastric cancer	Prof Robert Hawkins (The Christie NHS Foundation Trust) / Ryan Guest (Cellular Therapeutics Ltd)	Yes
A randomised Phase II study in metastatic melanoma to evaluate the effect of optimised cell production protocols. Gene modified T cells expressing an engineered TCR to recognise NY-ESO-1 cancer antigen	The Christie NHS Foundation Trust, Manchester, UK (Treatment centre and 6 other sites across EU); Cellular Therapeutics Ltd, UK - IMPD manufacturing	Phase 2. Target date for trial completion 2018				Engineered T cells	Autologous	Peripheral blood	Oncology	Advanced melanoma cancer	Prof Robert Hawkins (The Christie NHS Foundation Trust) / Ryan Guest (Cellular Therapeutics Ltd)	Yes
An open-label study of sipuleucel-T in European men with metastatic, castrate resistant prostate cancer	Dendreon	Phase 2 (open in UK, Austria, Holland)	2012	45	Yes	Antigen presenting cells (APCs)	Autologous	Blood	Oncology	Metastatic, castrate resistant prostate cancer	Todd Gumbleton, Lead Nurse, Barts Cancer Institute, Centre for Experimental Cancer Medicine, Queen Mary University of London, Todd.Gumbleton@bartshealth.nhs.uk	Yes

Patients with high-risk B cell precursor acute lymphoblastic leukaemia are treated with donor-derived EBV-specific cytotoxic T-lymphocytes transduced with the SFGαCD19-CD3 <sub>ζ</sub> retroviral vector following allogeneic haematopoietic stem cell transplantation	University College London	Phase 1/2	2012	75	Yes	CD8 cytotoxic T cell	Allogeneic	Peripheral blood mononuclear cells	Oncology	Acute lymphoblastic leukaemia	Dr Zahid Sattar, University College London, (z.sattar@ucl.ac.uk)	Yes
A Phase 1 study of adoptive transfer of autologous tumour antigen specific T cells with pre-conditioning chemotherapy and intravenous IL2 in patients with CD19 positive malignancy. Gene modified T cells expressing an engineered CAR to recognise the CD19 surface antigen	The Christie NHS Foundation Trust, Manchester, UK (Treatment centre); Cellular Therapeutics Unit, University of Manchester, UK - IMPD manufacturing	Phase 1. Target date for trial completion 2014				Engineered T cells	Autologous	Peripheral blood	Oncology	Advanced CD19 positive malignancies	Prof Robert Hawkins (The Christie NHS Foundation Trust) / Ryan Guest (Cellular Therapeutics Ltd)	Yes
Gene therapy for ADA-SCID. Autologous haematopoietic stem cells transplanted after modification with a retroviral vector expressing the human ADA gene	Great Ormond Street Hospital, London	Phase 1/2	2003	10	No	CD34+ stem cells	Autologous	Bone marrow or cord blood	Blood	Adenosine Deaminase Deficiency	Anne-Marie McNicol Clinical Trials Coordinator UCL Institute of Child Health London anne-marie.mcnicol@ucl.ac.uk	Yes
Gene therapy for Wiskott-Aldrich syndrome. Patient's own haematopoietic stem cells transplanted after modification with a lentiviral vector expressing the human Wiskott-Aldrich Syndrome protein gene	Genethon, France/Great Ormond Street Hospital, London	Phase 1/2	2010	5	Yes	CD34+ stem cells	Autologous	Bone marrow or peripheral blood following mobilisation	Blood	Wiskott-Aldrich syndrome	Anne-Marie McNicol Clinical Trials Coordinator UCL Institute of Child Health London anne-marie.mcnicol@ucl.ac.uk	Yes
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	Great Ormond Street Hospital, London	Phase 1/2	2011	10	Yes	CD34+ stem cells	Autologous	Bone marrow	Blood	X-linked severe combined immunodeficiency	Anne-Marie McNicol Clinical Trials Coordinator UCL Institute of Child Health London anne-marie.mcnicol@ucl.ac.uk	Yes
T cell suicide gene therapy following haploidentical stem cell transplantation. Infusion of transduced donor T cells expressing HSVTK in the haploidentical setting, to enable removal of donor T cells in the event of GvHD.	Great Ormond Street Hospital, London	Phase 1/2	2011	10	Yes	Donor T cells	Allogeneic	Peripheral blood	Blood	Patients with primary immunodeficiencies, haematological malignancies or metabolic disorders undergoing haploidentical transplant	Anne-Marie McNicol, Clinical Trials Coordinator, UCL Institute of Child Health, London (anne-marie.mcnicol@ucl.ac.uk)	Yes
Lentiviral gene therapy for ADA-SCID. Autologous haematopoietic stem cells transplanted after modification with a lentiviral vector expressing the human ADA gene	Great Ormond Street Hospital, London	Phase 1/2	2012	10	Yes	CD34+ stem cells	Autologous	Bone marrow or peripheral blood following mobilisation	Blood	Adenosine Deaminase Deficiency	Anne-Marie McNicol Clinical Trials Coordinator UCL Institute of Child Health London anne-marie.mcnicol@ucl.ac.uk	Yes

Randomised control trial to compare the effects of G-CSF and autologous bone marrow progenitor cells infusion in patients with ischaemic heart disease	Barts Health NHS Trust, Queen Mary University of London	Phase 2	2005	90	Recruitment completed, in follow up	Bone marrow mononuclear cells	Autologous	Bone marrow derived	Cardiovascular	Heart failure secondary to ischaemic heart disease	Professor Anthony Mathur, William Harvey Research Institute, Queen Mary University (a.mathur@qmul.ac.uk)	Yes
Autologous bone marrow derived mononuclear cells for acute myocardial infarction. Combines stem cell delivery with primary angioplasty within 5 hours post event	Barts Health NHS Trust, Queen Mary University of London, University College London	Phase 1/2	2007	70	Yes	Bone marrow mononuclear cells	Autologous	Bone marrow derived	Cardiovascular	Acute myocardial infarction	Professor Anthony Mathur, William Harvey Research Institute, Queen Mary University (a.mathur@qmul.ac.uk)	Yes
Autologous bone marrow derived mononuclear cells for dilated cardiomyopathy, delivered via intracoronary injection	Barts Health NHS Trust, Queen Mary University of London	Phase 1/2	2010	60	Yes	Bone marrow mononuclear cells	Autologous	Bone marrow derived	Cardiovascular	Dilated cardiomyopathy	Professor Anthony Mathur, William Harvey Research Institute, Queen Mary University (a.mathur@qmul.ac.uk)	Yes
Expanded adult haematopoietic stem cells for autologous infusion to patients with myocardial ischaemia	Imperial College London	Phase 1/2	2011	42	Yes	Expanded haematopoietic CD34+ stem cells	Autologous	Bone marrow	Cardiovascular	Localised myocardial dysfunction	Anne Bradshaw, Imperial College Healthcare NHS Trust (anne.bradshaw@imperial.nhs.uk; 0203 313 2056)	Yes
Stem cells in rapidly evolving active multiple sclerosis (STREAMS)	Imperial College London	Phase 2	2012	13	Yes	Mesenchymal stromal cells	Autologous	Bone marrow	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; 0203 313 2056)	Yes
Fetal brain tissue transplant for Parkinson's disease (TRANSEURO: An Innovative Approach for the Treatment of Parkinson's Disease)	Cambridge University	Phase 1/2	2012	20 transplanted patients, 20 controls	Yes, but all patients recruited to this study have been previously enrolled in the Transeuro observational study and all patients are selected from the observational study to take part in the transplant study	Fetal brain	Allogeneic		Neurological	Parkinson's disease	Danielle Daft, University of Cambridge	Yes
Autologous bone marrow derived CD34+ cells for ischemic stroke, administered within 7 days post event	Imperial College London	Phase 1	2007	10	Yes	Expanded CD34+ cells	Autologous	Bone marrow derived	Neurological	Ischemic stroke	Professor Nagy Habib, Imperial College London (nagy.habib@imperial.ac.uk)	Yes
ReN001: CTX neural progenitor cells for stroke disability	ReNeuron	Phase 1	2010	12	Yes	Neural	Allogeneic	Brain (cortex)	Neurological	Stroke disability	Dr John Sinden, ReNeuron Group plc (info@reneuron.com)	Yes
Autologous CD34+ haematopoietic cells for Crohn's disease	European Group for Blood and Marrow Transplantation (EBMT)	Phase 2/3	2006	45	No	CD34+ stem cells	Autologous	Bone marrow derived	Gastroenterology	Crohn's disease	Prof Hawkey, NDDC, West Block, E Floor, University Hospital, QMC, Nottingham NG7 2UH (cj.hawkey@nottingham.ac.uk) Trial Coordinator: Miranda Clark (astic@nottingham.ac.uk)	Yes
Repeated infusions of autologous CD133+ bone marrow stem cells for liver cirrhosis	Birmingham University, Edinburgh University	Phase 2	2009	81	Yes	CD133+ haematopoietic stem cells	Autologous	Bone marrow derived	Gastroenterology (Liver)	Liver cirrhosis	Dr Philip Newsome, University of Birmingham (p.n.newsome@bham.ac.uk)	Yes

Autologous expanded haemopoietic cells for liver insufficiency. Administered after 7 days expansion via the portal vein or hepatic artery	Imperial College London	Phase 1/2	2005		Yes	Expanded CD34+	Autologous	Derived by leukapheresis	Gastroenterology (Liver)	Liver insufficiency	Professor Nagy Habib, Imperial College London (nagy.habib@imperial.ac.uk)	Yes
Autologous cultured human limbal epithelium for limbal stem cell deficiency (ophthalmology)	Newcastle University	Phase 2	2012	24	Yes	Corneal	Autologous	Limbus	Ophthalmology	Limbal stem cell deficiency	Professor Francisco C Figueiredo, Newcastle University, UK	Yes
Corneal stem cells (allogeneic limbal epithelial stem cells on amniotic membrane)	Edinburgh University, Scottish National Blood Transfusion Service	Phase 1/2	2011	20	Yes	Corneal	Allogeneic	Limbus	Ophthalmology	Corneal stem cell deficiency	Margaret MacDonald, Research Nurse co-ordinator PAEP	Yes
Retinal pigment epithelial cell replacement for Stargardt's disease	Advanced Cell Technology	Phase 1/2	2011	12	Yes	Retinal pigment epithelium cell replacement derived from human embryonic stem cell	Allogeneic	Embryonic	Ophthalmology	Stargardt's disease	Dr. James Bainbridge, Moorefields Eye Hospital, London (j.bainbridge@ucl.ac.uk)	Yes
A Phase 3, multicenter, randomized, double-blind, parallel assignment study to assess the efficacy and safety of Reparixin in pancreatic islet transplantation	Dompé	Phase 3	2013	10	Yes	Pancreatic islets	Allogeneic	Deceased donor pancreas	Diabetes	Type 1 diabetes complicated by recurrent severe hypoglycaemia	Prof James Shaw, Institute of Cellular Medicine, Newcastle University	Yes
Autologous expanded CD34+ subset for diabetes. Administered after 21 days expansion	Imperial College London	Phase 1	2007		Yes	Expanded CD34+ subset	Autologous	Derived by leukapheresis	Diabetes	Diabetes type I or II	Professor Nagy Habib, Imperial College London (nagy.habib@imperial.ac.uk)	Yes
Biomedical and psychosocial outcomes of islet transplantation within the NHS clinical programme	Newcastle University	Experimental medicine follow-up of patients transplanted in UK clinical programme	2007	100	Yes	Pancreatic islets	Allogeneic	Deceased donor pancreas	Diabetes	Type 1 diabetes complicated by recurrent severe hypoglycaemia	Prof James Shaw, Institute of Cellular Medicine, Newcastle University	Yes
A comparison of Autologous Chondrocytes Implantation (ACI) versus existing techniques for knee cartilage repair	Keele University (Sponsor), Robert Jones & Agnes Hunt Orthopaedic Hospital NHS Trust (Host organisation)	Phase 2 (27 UK sites and 2 Norway sites)	2005	400	No	Chondrocytes	Autologous	Articular cartilage from non weight-bearing area of knee	Bone and Cartilage	Chondral/osteochondral defects	Professor James Richardson, Institute of Orthopaedics, Robert Jones & Agnes Hunt Orthopaedic Hospital, Oswestry, SY10 7AG	Yes
PACINO: Autologous cell therapy of fracture nonunion – cell phenotype as a predictor of outcome	University College London	Phase 2	2011	60	Yes	Mesenchymal stem Cells	Autologous	Bone marrow	Bone and cartilage	Bone regeneration and healing (orthopaedics)	Prof David Marsh (CI) Dr Michelle Korda (trial manager) michelle.korda@ucl.ac.uk	Yes
Autologous mesenchymal stem cells (MSCs) for knee meniscal repair. MSCs grown on biological scaffold for 2 weeks then surgically implanted	Azellon Cell Therapeutics	Phase 1/2	2012	10	Yes	Mesenchymal stem cells	Autologous	Bone marrow	Bone and cartilage	Knee meniscus repair	Professor Anthony Hollander, (CSO at Azellon); Univeristy of Bristol (anthony.hollander@bristol.ac.uk)	Yes