UK pre-clinical trial database (2 years from the clinic)

Project summary	Lead institution / company	Collaboration partners	Present stage of development and expected completion date	Next expected stage of development and expected start date	Cell type	Cell source	Autologous/ Allogeneic	Disease area	Indication	Contact
Design and optimization of cellular scaffolds for retinal ganglion cell replacement. Retinal ganglion cells derived from Müller stem cells are transiently attached to biodegradable materials, which are then grafted onto the inner retina to allow cell migration into the retinal ganglion cell layer.	University College London Institute of Ophthalmology	National Institute for Health Research	Preclinical Proof of Concept-Jul 2012	Late Preclinical-dependent on funding	Adult human retinal stem cells	Retina	Allogeneic	Ophthalmology	Glaucoma	Prof. G Astrid Limb, UCL Institute of Ophthalmology, g.limb@ucl.ac.uk
Preclinical validation of the regenerative potential of retinal ganglion cells (RGC) derived from Muller stem cells. Müller stem cells differentiated into retinal ganglion cells are transplanted onto the inner retina of experimental models of retinal ganglion cell damage. Transplanted cells proved to partially restore retinal ganglion cell function in these models.	University College London Institute of Ophthalmology	Medical Research Council	Preclinical Proof of Concept-Oct 2012	Late Preclinical-dependent on funding	Adult human retinal stem cells	Retina	Allogeneic	Ophthalmology	Glaucoma, retinitis pigmentosa and Age-related macular degeneration (AMD)	Prof. G Astrid Limb, UCL Institute of Ophthalmology, g.limb@ucl.ac.uk
Development of a synthetic biodegradable cell carrier membrane for the transplantation of cultured cells or freshly excised autologous tissue (limbal segments or oral mucosa) to restore sight to eyes where the cornea has been damaged by chemical injury or burns.	LV Prasad Eye Institute with University of Sheffield	Wellcome Trust	Late Preclinical-2014	Clinical Trial Ph1/2-2014	Corneal plus scaffold	Cornea	Autologous	Ophthalmology	Cornea	Prof. Sheila MacNeil
Modulation of limbal niche stiffness to regulate stem cell differentiation. A pharmacological approach is to be established which will improve stem cell engraftment.	University of Reading	Imperial College	Preclinical Proof of Concept- 2015	Clinical Trial Ph1/2-2016	Adult epithelial origin	Corneal limbus	Allogeneic	Ophthalmology	Ophthalmology (limbal stem cell deficiency)	Dr. Che Connon, c.j.connon@reading.ac.uk
Corneal limbal stem cells on a matrix.	University College London Institute of Ophthalmology	TAP Biosystems	Preclinical Proof of Concept		Corneal		Autologous	Ophthalmology	Corneal replacement	Prof. Julie T Daniels
Human embryonic stem cells for Diabetic retinopathy.	University College London Institute of Ophthalmology		Late Preclinical-2013	Clinical Trial Ph1/2	Endothelial progenitors	Human embryonic stem cells	Allogeneic	Ophthalmology	Diabetic Retinopathy (Macular ischemia sub population)	Prof. Julie T Daniels

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Cell therapy for the vasodegenerative stages of diabetic retinopathy. Mechanisms underlying endothelial progenitor cell (EPC) defects in diabetes are being evaluated.	Queen's University Belfast	Medical Research Council	Preclinical Proof of Concept- 2014		Endothelial progenitor cells (circulating angiogenic cells, myeloid angiogenic cells)	Peripheral blood and cord blood	Allogeneic	Ophthalmology	Diabetic retinopathy	Prof. Alan Stitt, a.stitt@qub.ac.uk
Endothelial progenitor cells and outgrowth endothelial cells for diabetic retinopathy. Role of bone marrow derived endothelial progenitor cells and outgrowth endothelial cells in vasoreparation.	Queen's University Belfast	Medical Research Council	Early Preclinical-2014		Endothelial progenitor, outgrowth endothelial cell (OEC)	Peripheral blood and cord blood	Autologous	Ophthalmology	Diabetic retinopathy	Prof. Alan Stitt, a.stitt@qub.ac.uk
##	Queen's University Belfast	Jules Thorn Trust; National University of Singapore; University of Oxford	Enabling / Platform Research-2015		Outgrowth endothelial cells (OECs)	Peripheral blood and cord blood	Autologous	Ophthalmology	Ischaemic retinopathies: Central Vein Occlusion & Branch Retinal Vein Occlusion (CVO and BRVO)	Prof. Alan Stitt, a.stitt@qub.ac.uk
Stromal stem cells for diabetic complications. Using a uniquely sorted stromal stem cell population that can induce vascular repair. This is an FP-7 grant with a large collaborative partnership. We are conducting the diabetic retinopathy component.	Queen's University Belfast	REMEDI (Galway); Orbsen Therapeutics; Lonza; Klinikum der Ludwigs-Maximilians Universität Muenche; Universitaetsklinikum Berlin	Late Preclinical-2016		Stromal stem cells (mesenchymal stem cells)	Bone marrow; cord blood	Allogeneic	Ophthalmology	Diabetic retinopathy	Prof. Alan Stitt, a.stitt@qub.ac.uk
ReN003 for retinitis pigmentosa. Human retinal progenitor cell (hRPC) line for retinitis pigmentosa.	ReNeuron	University College London Institute of Ophthalmology, Schepens Eye Research Institute	Late Preclinical-2014	Clinical trial Ph1/2-2014	Human retinal progenitor cells	Fetal eyes (retina)	Allogeneic	Ophthalmology	Retinitis pigmentosa	Dr. Sara Patel, Sara-Patel@reneuron.com
Retinal pigment epithelium cells derived from hECSs on membrane.	Pfizer Neusentis	University College London Institute of Ophthalmology; Moorfields Eye Hospital; University College London Cells for sight Facility manufacturing hES derived RPE Cells for Pfizer.	Late Preclinical-2014	Clinical trial Ph1/2-2014	Retinal pigment epithelium cells	Embryonic stem cells	Allogeneic	Ophthalmology	Age related macular degeneration	Dr. Paul Whiting, paul.whiting@pfizer.com

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Biodegradable Magnetic Stent for Coronary Artery Luminal Regeneration (BIOMAGSCAR).	University College London	Qualimed, University College London, University of Eastern Finland (UEF), MI, EURAM, Queen Mary's University London, Yale	Preclinical Proof of Concept-2016	Clinical Trial Ph1-2016	Bone marrow cells	Bone Marrow	Autologous	Cardiovascular	Coronary Artery Disease	Prof. John Martin, j.martin@ucl.ac.uk; Prof. Anthony Mathur, a.mathur@qmul.ac.uk
Human bone marrow-derived MSCs: disruption of extracellular matrix/ growth factor receptor signals induces endothelial fate.	University of Manchester	UKRMP Hub 'Engineering and exploiting the stem cell niche'	Preclinical Proof of Concept-Jun 2014	Late Preclinical-Mid 2014	Mesenchymal Stem Cells	Bone marrow	Allogeneic	Cardiovascular	Neovascularisation of ischaemic tissues (non-healing wounds, cardiac post-myocardial infarct etc.)	Prof. Cay Kielty, Faculty of Life Sciences, University of Manchester, M13 9PT, UK.
Clinical transplantation of endothelial cells derived from human embryonic stem cells.	University of Glasgow	Roslin Cells, Medical Research Council	Late Preclinical	Clinical Trial Ph I/II-2015	Endothelial Cells	Human embryonic stem cells	Allogeneic	Cardiovascular	Peripheral limb ischemia	Andrew H Baker, andrew.h.baker@glasgow.ac.uk
Adult mesenchymal stem cell (MSC) applications in regenerative medicine (regulation by extracellular matrix niche).Human bone marrow-derived MSCs: disruption of extracellular matrix/ growth factor receptor signals induces endothelial fate.	University of Manchester	UKRMP Hub 'Engineering and exploiting the stem cell niche'	Preclinical Proof of Concept-Jun 2014	Late Preclinical-Mid 2014	Mesenchymal stem cells	Bone marrow	Allogeneic	Cardiovascular	Neovascularisation of ischaemic tissues (non-healing wounds, cardiac post-myocardial infarct etc.)	Prof. Cay Kielty, Faculty of Life Sciences, University of Manchester, M13 9PT, UK.
ReN009: for limb ischemia. CTX cell line for critical limb ischemia. 9 patient Ph 1 study being planned.	ReNeuron		Late Preclinical-2014	Clinical Trial Ph1/2-2014	Human neural stem cells	Fetal cortex	Allogeneic	Cardiovascular	Limb Ischemia	Dr. Paul Stroemer, Paul-Stroemer@reneuron.com
Autologous bone marrow derived cells enriched for angiogenic potential for cardiac repair. Pericytes harvested from veins or hearts delivered in models of limb or myocardial ischaemia, with standard operating procedure transferred to clinical grade facilities.	Bristol University	British Heart Foundation	Preclinical Proof of Concept-2015	Clinical Trial Ph1/2-2015	Pericyte and Cardiac progenitor cells	Vein and heart	Both	Cardiovascular	Cardiac repair	Prof. Paolo Madeddu, University of Bristol, email madeddu@yahoo.com mdprm@bristol.ac.uk

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Autologous bone marrow derived cells enriched for angiogenic potential for cardiac repair. BM stem cells from patients with MI participating to the Transact trials sorted on the basis of their migratory activity.	Bristol University	Medical Research Council	Preclinical Proof of Concept-2016		Endothelial progenitor Cell	Bone marrow	Autologous	Cardiovascular	Cardiac repair (post acute myocardial infarction)	Prof. Paolo Madeddu, University of Bristol, email madeddu@yahoo.com mdprm@bristol.ac.uk
T-Control Trial. This project aims to evaluate the safety and feasibility of using cord blood regulatory T cells to treat refractory steroid GvHD in transplanted patients.	Anthony Nolan in the UK and Wurzburg University in Germany	Stage, SME, Germany and LUMC, Leiden, Netherlands	Late Preclinical-May 2014	Clinical Trial Ph1-May 2014	Regulatory T cells	Umbilical cord blood	Allogeneic	Oncology	Steroid resistant acute Graft versus Host Disease after haematopoietic stem cell transplantation	Prof. MaDr.igal a.maDr.igal@ucl.ac.uk or Dr. Saudemont aurore.saudemont@anthonynolan.org
MSCs genetically engineered to express TNF related apoptosis ligand (TRAIL) as a treatment for lung cancer.	University College London	Medical Research Council	Preclinical Proof of Concept-2013	Clinical Trial Ph1/2-2014	Mesenchymal stem cells	Bone marrow	Allogeneic	Oncology	Oncology - lung cancer and pleural mesothelioma	Dr. Sam Janes, Lungs for Living Research Centre, University College London. S.janes@ucl.ac.uk
Development of Adoptive immunotherapy of EBV-associated Malignancies by Retroviral LMP2-TCR Gene Transfer.	University College London	Medical Research Council	Late Preclinical-2014	Clinical Trial Ph 1-2014	T cell	Peripheral blood mononuclear cells	Autologous	Oncology	Oncology - EBV lymphoma, EBV nasopharyngeal carcinoma	Prof. Hans Stauss and Dr. Emma Morris, Institute of Immunity and Transplantation, University College London, h.stauss@ucl.ac.uk.
T cell receptor gene therapy of multiple myeloma targeting cancer-testis antigens. Genetic engineering of patient T cells with the genes encoding a tumour-reactive cancer-testis antigen specific TCR.	University of Birmingham	Leukaemia & Lymphoma Research	Preclinical Proof of Concept- 2016	Late Preclinical-2016	T cell	Peripheral blood	Autologous	Oncology	Multiple myeloma	Dr. Gavin Bendle, School of Cancer Sciences, University of Birmingham (g.bendle@bham.ac.uk)
T cell receptor gene transfer to target Epstein-Barr virus-associated human cancers. T cell receptor gene transfer to target Epstein-Barr virus-associated human cancers using TCRs cloned from CD8+ or CD4+ virus-specific T cell effectors.	Birmingham University		Preclinical Proof of Concept- 2014	Clinical Trial Ph1-Unsure	T cell	Peripheral blood	Autologous	Oncology	EBV+ tumours	Dr. Steve Lee, University of Birmingham (s.p.lee@bham.ac.uk)

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Project summary	Lead institution / company	Collaboration partners	Present stage of development and expected completion date	Next expected stage of development and expected start date	Cell type	Cell source	Autologous/ Allogeneic	Disease area	Indication	Contact
Genetic modification of T cells to target the tumour vasculature. Engineering human T cells to target the tumour vasculature through expression of a Chimeric antigen receptor.	Birmingham University		Preclinical Proof of Concept- 2015	Clinical Trial Ph1-2015	T cells	Peripheral blood	Autologous	Oncology	Multiple common solid tumours	Dr. Steve Lee, University of Birmingham (s.p.lee@bham.ac.uk)
Tumour lysate primed NK cells for multiple myeloma	University College London	University of Southampton / Cell Therapy Catapult / Medical Research Council	Late Preclinical-2013	Clinical trial Ph1/2-2014	NK cells		Autologous	Oncology	Multiple myeloma	Prof. Mark Lowdell, m.lowdell@ucl.ac.uk / K.H.Orchard@soton.ac.uk
Neural progenitor cells from human embryonic stem cells (hESCs) for neurological conditions. GMP culture protocols for hESC derived neural progenitor cells for Huntington's disease.	Cardiff University	Medical Research Council / National Institute for Social Care and Health Research	Preclinical Proof of Concept- 2017		Neuronal derived from human embryonic stem cells (hESCs) and human induced pluripotent stem cells (hiPSCs)	Human embryonic stem cell (hESC) and human induced pluripotent stem cells (hiPSC) lines	Allogeneic	Neurological	Neurological (Huntington's disease)	Prof. S.B Dunnett, Brain Repair Group, School of Biosciences, Cardiff University, Museum Avenue, Cardiff CF10 3AX, South Wales, UK (dunnett@cf.ac.uk)
SWIFT : Viability, specificity and yields of clinical grade primary and expanded human fetal cells for neural disorder. Preparation for pilot trial on the safety and feasibility of fetal neural tissue transplantation, including GMP culture protocols.	Cardiff University	Medical Research Council / National Institute for Social Care and Health Research	Late Preclinical-2016	Clinical Trial Ph1/2-2015	Neuronal derived from embryonic brain, fetal neural progenitors (FNPs), hESCs and hiPSCs	Human embryonic brain (7-11 wk.) and cell lines	Allogeneic	Neurological	Neurological (Parkinson's and Huntington's)	Prof. S.B Dunnett, Brain Repair Group, School of Biosciences, Cardiff University, Museum Avenue, Cardiff CF10 3AX, South Wales, UK (dunnett@cf.ac.uk)
Repair-HD. Preparation for first in man trials on the safety and feasibility of human stem cell-derived striatal tissue transplantation in Huntington's disease.	Cardiff University	EU FP7 collaboration incl. Cardiff, Edinburgh, Manchester, Paris, Munster, Turin	Late Preclinical-2017	Clinical Trial Ph1/2-2017	Striatal neurons differentiated from clinical grade stem cell lines	Clinical grade endothelial stem cell lines (Roslin RC9 ...) and newly generated fetal-derived iPS cells (sourced from CFTB)	Allogeneic	Neurological	Neurological (Huntington's disease)	Prof. S.B Dunnett, Brain Repair Group, School of Biosciences, Cardiff University, Museum Avenue, Cardiff CF10 3AX, South Wales, UK
Development of decellurisation/recellurisation methods for production of a tissue engineered oesophagus.	University College London (UCL) / Great Ormond Street Hospital, London (GOSH) / Royal Free Hospital, London (RFH) / University College London Hospital (UCLH)	UK Stem Cell Foundation	Late Preclinical-2016	Clinical trial Ph1/2-2016	Mesenchymal stem cells/ epithelia	Muscle / buccal mucosa biopsies	Autologous (cells) / allogeneic (scaffold)	Gastroenterology	Congenital oesophageal abnormalities, oesophageal atresia, oesophageal injury/loss	Prof. Martin Birchall, Dr. Paolo De Coppi, Dr. Mark Lowdell m.birchall@ucl.ac.uk / p.decoppi@ucl.ac.uk / m.lowdell@ucl.ac.uk

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Adrenocortical stem cells. Project aims to use adrenocorticotrophic hormone (ACTH) to stimulate stem cells back into activity, potentially restoring adrenal gland function.	Newcastle University		Preclinical proof of concept		Endogenous adrenocortical stem cells		Autologous	Gastroenterology		Prof. Simon Pearce
Tissue engineered autologous stem cell seeded bowel for replacement. Develop a tissue engineered bowel for treatment of short bowel syndrome. Utilises decellularisation/recellularisation technology developed at Northwick Park.	Videregen Ltd	Northwick Park Institute for Medical Research	Preclinical Proof of Concept- 2015	Late preclinical-2015	Mesenchymal stem cells and bowel cells	Bowel and bone marrow/adipose tissue	Autologous	Gastroenterology	Short bowel syndrome (Crohn's' disease, necrotising enterocolitis)	Dr. Steve Bloor, Videregen Ltd. Email: stevebloor@videregen.com
RegenVox 1 Preclinical development and animal testing of engineered larynx.	University College London	Northwick Park Institute for Medical Research/Medical Research Council	Preclinical Proof of Concept-2013	Clinical trial Ph1/2-2014	Mesenchymal stem cell / epithelia	Bone marrow and tracheal biopsies	Autologous	Respiratory	Ca or traumatic injury to larynx	Prof. Mark Lowdell, m.birchall@ucl.ac.uk / m.lowdell@ucl.ac.uk
RegenVox 2 Clinical trial of engineered larynx.	University College London	Northwick Park Institute for Medical Research/Medical Research Council	Late Preclinical-2014	Clinical trial Ph1/2-2014	Mesenchymal stem cell / epithelia	Bone marrow and tracheal biopsies	Autologous	Respiratory	Ca or traumatic injury to larynx	Prof. Mark Lowdell, m.birchall@ucl.ac.uk / m.lowdell@ucl.ac.uk
Tissue engineered autologous stem cell seeded trachea replacement. Development of a tissue engineered trachea replacement using a decellularised human trachea, seeded with autologous bone marrow derived MSCs and airway epithelial cells. For the treatment of severe structural airway diseases.	Videregen Ltd	Cell Therapy Catapult, UCL, Royal Free, NHSBT and Northwick Park Institute for Medical Research	Late Preclinical-2014	Clinical Trial Phase I/II-Q1 2015	Autologous mesenchymal stem cells and epithelial cells	Bone marrow and airway	Autologous	Respiratory	Structural airway diseases	Dr. Steve Bloor, Videregen Ltd, stevebloor@videregen.com
Generation and expansion of antigen-specific regulatory T-cells for the treatment of autoimmune hepatitis. Autologous expanded antigen-specific T-reg population to control effector immune responses.	King's College London	Medical Research Council	Preclinical Proof of Concept-2014	Late Preclinical-2016	Regulatory T-cells	Peripheral blood	Autologous	Immunology	Immunology - Autoimmune hepatitis	Dr. Maria Serena Longhi, Institute of Liver Studies, King's College London School of Medicine at King's College Hospital; maria.longhi@kcl.ac.uk

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Polyclonal Tregs to support Kidney transplantation. Polyclonal Tregs to support immune tolerance to transplant; n=12 (Treg treatment) patients planned in Ph1/2 study.	University of Regensburg	King's College London, University of Oxford, Charite Berlin, HSR Milano, UCSF, University of Wisconsin, University of Loughborough, ITERTUN-INSERM Nantes	Late Preclinical-Feb 14	Clinical Trial Ph1/2-Apr 14	Treg-cells	Whole blood	Autologous	Immunology	Kidney Transplantation	Prof. Giovanna Lombardi, Kings College London, UK, Giovanna.lombardi@kcl.ac.uk
Red blood cells derived from pluripotent stem cell lines. BloodPharma 1 demonstrated that RBCs can be differentiated from hESC / hiPSC using a feeder and xeno free GMP-grade culture system. BloodPharma 2 aims to optimise the biology and engineering in order to conduct first in man clinical study and create a platform for further investment.	Scottish National Blood Service	Universities of Edinburgh, Glasgow, Cambridge, Loughborough, Bristol, NHS Blood and Transplant, Irish Blood Transfusion Service, Rosin Cells, Cell Therapy Catapult	Preclinical Proof of Concept- 2017	Clinical Trial Ph1/2-2017	Red blood cells	Human embryonic stem cells (hESCs)/ human induced pluripotent stem cells (hiPSCs)	Allogeneic	Blood		Prof. Marc Turner
Platelets derived from iPS. iPSC derived megakaryocytes now in preclinical studies for platelet production.	University of Cambridge	NHS Blood and Transplant	Preclinical Proof of Concept- 2014	Late preclinical and first-in-man volunteer study-2015	Platelet and megakaryocytes	Induced pluripotent stem cells (iPSCs)	Allogeneic	Blood	Blood (platelets) for transfusion	Dr. Cedric Ghevaert, Haematology Department, Cambridge Blood Centre, Long Road, Cambridge CB2 0PT 01223 548082, cg348@cam.ac.uk
##	The Royal Veterinary College	Prof. A. Carr, University of Oxford; Prof. Dennis McGonagle and Dr. Elena Jones, University of Leeds; funded by the MRC	Preclinical Proof of Concept- Mar 2015	Clinical trial Ph1/2-2015	Mesenchymal stem cells	Bone marrow and synovium	Autologous	Bone and Cartilage	Tendon healing	Prof.essor Roger K.W. Smith, Dept. Clinical Sciences and Services, The Royal Veterinary College
Brown Adipose. Adult adipose derived MSCs differentiated into brown adipocytes.	Plasticell		Preclinical Proof of Concept- 2014	Late Preclinical-2014	Brown Adipose	Adipose tissue	Autologous	Diabetes	Treatment of obesity and diabetes	Dr. Yen Choo, yen@plasticell.co.uk
Developing an ex vivo gene therapy for keratin-associated inherited blistering skin diseases using epidermal stem cells and the novel TALE nuclease technology. Keratinocyte stem cells from EBS patients are treated ex vivo with TALENs to disrupt the mutant allele before autologous grafting.	Newcastle University		Preclinical Proof of Concept-2014/2015	Late Preclinical-2014/2015	Keratinocyte stem cells	Skin	Autologous	Dermatology/ wound healing	Epidermolysis bullosa	Dr. Julia Reichelt, julia.reichelt@ncl.ac.uk

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Polyclonal Tregs to support immune tolerance to liver transplant; Stage 1- n=6 (Treg treatment) Stage 2- n=17 (Treg treatment) patients planned in Ph1/2 study.	King's College London	Medical Research Council	Late Preclinical-Feb 14	Clinical trial Ph1/2-Apr 14	Treg-cells	Whole blood	Autologous	Liver	Liver transplantation	Prof. Giovanna Lombardi,Kings College London, UK, Giovanna.lombardi@kcl.ac.uk
hESC derived otic neuroprogenitors for deafness, examination of function in animal models.	Sheffield University	Medical Research Council, Action on Hearing Loss	Preclinical Proof of Concept- 2014	Late Preclinical-Sep 2014	Otic	Human embryonic stem cells (hESCs)/ human induced pluripotent stem cells (hiPSCs)	Allogeneic	Other	Deafness	Dr. Marcelo Rivolta, Centre for Stem Cell Biology, University of Sheffield, m.n.rivolta@sheffield.ac.uk
Development of process for procurement, testing and therapeutic banking of amniotic fluid stem cells.	University College London	Great Ormond Street Hospital Biomedical Research Council	Enabling / Platform Research-2014	Clinical trial Ph1/2-2015	Amniotic Fluid Stem Cells	Amniotic fluid	Allogeneic	Other	Necrotising enterocolitis	Dr. Mark Lowdell, p.decoppi@ucl.ac.uk / m.lowdell@ucl.ac.uk / a.david@ucl.ac.uk