Cell Therapy Catapult Preclinical Database 2015

Name	Organisation	Project Title	Project Summary	Funding Source(s)	Stage of Development	Expected completion Date	Cell Type	Cell Source	Autologous or Allogeneic	Genetic Modification & Method of Modification	Disease Area	Clinical Indication
Che Connon	Newcastle University	Scaling up of ambient cell storage, using hydrogel encapsulation.	Building upon previous data to scale up encapsulation of stem cells for storage/transport at room temperature in collaboration with Bioprocessing community.	BBSRC; EPSRC	Early preclinical	Beginning of 2016	Mesenchymal stem cells, amniotic stem cells (range of stem cells)	-	Allogeneic	No	All	All
Dr Cedric Ghevaert	University of Cambridge	Platelets derived from induced pluripotent stem cells.	Induced pluripotent stem cell-derived megakaryocytes now in preclinical studies for platelet production.	MRC; NIHR; Wellcome Trust	Early preclinical	2017	platelets and megakaryocytes	induced pluripotent stem cells	Allogeneic	Yes - combination of lentiviral transduction and genome editing with CRISPR	Haematology	Blood (platelets) for transfusion
Dr Steve Lee	University of Birmingham	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 T-cell receptors, cloned from CD8+ or CD4+ virus-specific T-cell effectors.	Cancer Research UK	Early preclinical	End of 2015	T-cells	Peripheral blood	Autologous	Yes - retroviral gene transfer	Oncology	EBV+ tumours
Hanns Lochmuller & Chris Denning	Newcastle University	Induced pluripotent stem cells in Duchenne Muscular Dystrophy.	New in vitro models of Duchenne Muscular dystrophy by induced pluripotency in patient biopsies and gene knockdown in human embryonic stem cells. ongoing project to investigate the characteristics of dystrophic cardiomyocytes derived from Duchenne Muscular Dystrophy patient biopsies after derivation from induced pluripotent stem cells.	MRC	Early preclinical	End of 2015	Induced pluripotent stem cells	-	Autologous	No	Cardiovascular	n/a
Hanns Lochmuller & Jenny Morgan	Newcastle University	Genetically modified stem cells in Duchenne Muscular Dystrophy.	Genetically modified stem cells in Duchenne Muscular Dystrophy. Lentivirally-mediated stem cells to treat Duchenne muscular Dystrophy. ongoing project to define the optimal stem cell for repopulating dystrophic muscle on systemic application following ex vivo gene correction.	MRC	Early preclinical	End of 2015	CD34+ and/or CD133+ stem cells	-	Autologous	Yes - lentiviral transduction	Neurology	Duchenne Muscular Dynstrophy
Majlinda Lako	Newcastle University	Development of synthetic retina	Exploiting the power of human induced pluripotent stem cells to generate synthetic retina in vitro for cell based therapies, drug discovery and disease modelling.	EU	Early preclinical	-	Retinal ganglion cells	induced pluripotent stem cells	-	No	Opthalmology	Blindness caused by age related degeneration of retina or inherited retinal disorders
Majlinda Lako	Newcastle University	Induced pluripotent cell-based disease model for age-related macular degeneration.	Assessing the feasibility of induced pluripotent stem cells to provide a disease model for age-related macular degeneration.	Other	Early preclinical	-	Retinal pigment epithelial cells	induced pluripotent stem cells	-	No	Opthalmology	Blindness caused by age related degeneration of retina
Majlinda Lako	Newcastle University	PRPF31 patient specific induced pluripotent stem cells	Improving our understanding of autosomal dominant retinitis pigmentosa, using PRPF31 patient specific induced pluripotent stem cells .	Other	Early preclinical	-	Retinal ganglion cells	induced pluripotent stem cells	-	No	Opthalmology	Blindness caused by inherited retinal disorders
Majlinda Lako	Newcastle University	Stem cells for biological assays of novel drugs and predictive toxicology	This is aimed at deriving human induced pluripotent stem cell lines from 500 patients with neurodegenerative disorders.	EU	Early preclinical	2017	Neural progenitor cells	induced pluripotent stem cells	-	No	Neurology	Neurodegeneration
Prof Mark Lowdell	University College London	Tumour lysate primed naturak killer cells for multiple myeloma.	Tumour lysate primed natural killer cells for multiple myeloma.	MRC	Early preclinical	-	Natural killer cells		Autologous	No	Oncology	Multiple myeloma
Prof Peter Jones	King's College London	Using mesenchymal stem cells to improve islet transplantation outcome	Co-culturing and co-transplanting islets with mesenchymal stem cells to improve survival and function of islet grafts as a treatment for Type 1 diabetes.	Diabetes UK	Early preclinical	2015	Mesenchymal stem / stromal cells	Adipose, bone marrow, pancreas	Allogeneic	No	Diabetes	Type 1 diabetes
Prof S.B Dunnett	Cardiff University	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.	MRC	Early preclinical	-	Embryonic brain, fetal neural progenitors, human embryonic stem cells and induced pluripotent stem cells	Human embryonic brain (week 7-11) and cell lines	Allogeneic	No	Neurology	Parkinson's and Huntington's Disease

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Yen Choo	Plasticell	Cord blood heamatopoietic stem cells	Adult hematopoeitic stem cells expanded ex vivo	Innovate UK	Early preclinical	End of 2015	CD34+ and CD133+ stem cells	Cord blood	Allogeneic	No	Oncology	Re-population of immune system following chemo/radio therapy
David Choi	UCL	Olfactory ensheathing cells for Brachial Plexus repair.	Preclinical development of olfactory ensheathing cells for use in Brachial Plexus repair. Work will include optimisation of cell population and manufacturing process.	-	Early preclinical	-	Olfactory ensheathing cells	Olfactory mucosa of nose	Autologous	No	Neurology	Brachial Plexus repair
Kevin Docherty	Aberdeen Univeristy	IsletCTS	To develop a scalable, cost-effective process for the production of islet cells from pancreatic exocrine tissue.	-	Early preclinical	-	Pancreatic islets	Pancreatic exocrine material	Allogeneic	No	Diabetes	Type 1 diabetes
Stefano Pluchino	Cambridge University	Induced neural stem cells for progressive Multiple Sclerosis	Development of patient-specific induced neural stem cells as a therapeutic for progressive Multiple Sclerosis	Multiple Sclerosis Societies; private investment / venture capital.	Early preclinical	End of 2016	Directly reprogrammed fibroblasts	Skin (or other accessible sources)	Autologous	Yes - Sendai virus	Neurology	Multiple Sclerosis, stroke, spinal cord injury, traumatic brain injury
Jenny Southgate	York University	Composite Cystoplasty	Development of a novel approach for bladder augmentation utilising autologous urothelium attached to vascularised demucosalised bowel.	MRC	Early preclinical	End of 2015	Urothelial cells	Bladder	Autologous	No	Urology	Bladder Exstrophy
Andrew Baker	Glasgow University	Clinical transplantation of endothelial cells derived from human embryonic stem cells.	Generating human endothelial cells from human embyronic stem cell lines under GMP-compliant conditions. Final protocol optimisation and validation, safety studies, efficacy and biodistribution studies.	MRC	Mid preclinical	End of 2016	Endothelial Cells	Human embryonic stem cells	Allogeneic	No	Cardiovascular	Peripheral limb ischemia
Dr Maria Serena Longhi	King's College London	Generation and expansion of antigen-specific regulatory T-cells for the treatment of autoimmune hepatitis.	Autologous expanded antigen-specific T-regulatory cell population to control effector immune responses.	MRC	Mid preclinical	End of 2015	Regulatory T-cells	Peripheral blood	Autologous	No	Immunology	Autoimmune hepatitis
Dr Steve Bloor	Videregen Ltd	Tissue engineered bowel	Develop a tissue engineered bowel for treatment of short bowel syndrome. Utilises decellularisation/recellularisation technology developed at Northwick Park.	Private investment / venture capital	Mid preclinical	End of 2015	Mesenchymal stem / stromal cells	Bowel; bone marrow; adipose tissue	Autologous	No	Gastroenterology	Short bowel syndrome (Crohn's' disease, necrotising enterocolitis)
Dr Steve Lee	Birmingham University	CLEC14A-targeted T-cells	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.	MRC	Mid preclinical	Mid 2015	T-cells	Peripheral blood	Autologous	Yes - CAR gene transfer	Oncology	Multiple common solid tumours
Marc Turner	Scottish National Blood Service	Red blood cells derived from pluripotent stem cell lines.	BloodPharma 1 demonstrated that RBCs can be differentiated from human embryonic stem cells and induced pluripotent stem cells, 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.	Wellcome Trust; Scottish Funding Council	Mid preclinical	2016	Red blood cells	Human embyronic stem cells & induced pluripotent stem cells	Allogeneic	No	Haematology	Beta thalassaemia
Prof Mark Lowdell	University College London	RegenVox 1	Preclinical development and animal testing of engineered larynx.	MRC	Mid preclinical	-	Mesechymal stem / stromal cells, epithalia	Bone marrow and tracheal biopsies	Autologous	No	Respiratory Medicine	Traumatic injury to larynx
Prof S.B Dunnett	Cardiff University	Neural progenitor cells from human embryonic stem cells (hESCs) for neurological conditions.	GMP culture protocols for human embryonic stem cell-derived neural progenitor cells for Huntington's disease.	MRC	Mid preclinical	-	Neural stem cells	Human embryonic stem cells & human induced pluripotent stem cells.	Allogeneic	No	Neurology	Huntington's Disease
Prof. Alan Stitt, Dr Reinhold Medina and Prof Noemi Lois	Queen's University Belfast	Vascular stem cell therapy for ischaemic retinopathy.	Outgrowth endothelial cells from patients with central vein and branch vein ischaemia. Also study of olfactory endothelial cell senescence ex vivo with a view to allowing expansion of a patients cells to allow autologous therapy.	Jules Thron Trust	Mid preclinical	-	Outgrowth endothelial cells / endothelial colony-forming cells	Peripheral blood and cord blood	Autologous	No	Opthalmology	Ischaemic retinopathies: Central Vein Occlusion & Branch Retinal Vein Occlusion

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Prof. G Astrid Limb	University College London Institute of Ophthalmology	Preclinical validation of the regenerative potential of retinal ganglion cells 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.	MRC	Mid preclinical	-	Retinal cells	Retina	Allogeneic	No	Opthalmology	Glaucoma, retinitis pigmentosa and Age-related macular degeneration
Sue Kimber	University of Manchester	Pluripotent stem cell-derived Cartilage Cells	-	MRC; ARUK; EU	Mid preclinical	Late 2015	Chondrocytes	Human embryonic stem cells	Allogeneic	No	Orthopaedics	Osteoarthritis, sports injury and similar conditions
Mark Kotter	Cambridge University	Olfactory ensheathing cells for Spinal Cord Repair	Optimisation of olfactory ensheathing cells for spinal cord repair to allow a more consitent patient response through definition of the cell population and development of a manufacturing proces which produces the same population of cells from each source.	-	Mid preclinical	-	Olfactory ensheathing cells	Olfactory bulb	Autologous	No	Neurology	Spinal cord injury
Dr. Georgina Ellison	Kings College London	c-kit postive cardiac stem cells in heart failure	c-kit postive cardiac stem cells, resident in the heart, have been demonstrated in animal models to regenerate cardiomyocytes and vasculature. We are now looking to translate this work into the clinical setting in order to perform a first-in-man study for patients with heart failure.	EU	Mid preclinical	-	c-kit positive Cardiac Stem Cells	Adult myocardium	Allogeneic	No	Cardiovascular	Heart failure
Prof. Paolo De Coppi	University College London	Tissue-engineered oesophagus	Development of decellurisation/recellurisation methods for production of a tissue-engineered oesophagus.	UK Stem Cell Foundation	Late Preclinical	End of 2016	Muscle progenitor cells / epithelial progenitor cells	Muscle and oral biopsies	Autologous	No	Gastroenterology	Congenital deformity, oesophageal atresia
Dr Paul Whiting	Pfizer Neusentis	Retinal pigment epithelium cells	Retinal pigment epithelium cells derived from human embryonic stem cells on membrane.	-	Late Preclinical	-	Retinal pigment epithelium cells	Human embryonic stem cells	Allogeneic	No	Ophthalmology	Age-related macular degeneration
Prof Sam Janes	University College London	Mesenchymal stem cell-TRAIL	Mesenchymal stem cells genetically engineered to express TNF related apoptosis ligand (TRAIL) as a treatment for lung cancer.	MRC	Late Preclinical	Mid 2017	Mesenchymal stem / stromal cells	-	Allogeneic	Yes - engineered to express TNF related apoptosis ligand (TRAIL)	Oncology	Non-small cell lung cancer (adenocarcinoma)
Dr Steve Bloor	Videregen Ltd	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 mesenchymal stem cells and airway epithelial cells. For the treatment of severe structural airway diseases.	Innovate UK; private investment / venture capital	Late Preclinical	-	Autologous mesenchymal stem cells and epithelial cells	Bone marrow and airway	Autologous	No	Respiratory Medicine	Structural airway diseases
Prof Madrigal & Dr Saudemont	Anthony Nolan in the UK and Wurzburg University in Germany	T-Control Trial	This project aims to evaluate the safety and feasibility of using cord blood regulatory T-cells to treat GvHD in transplanted patients.	EU	Late Preclinical	Beginning of 2015	Regulatory T-cells	Cord blood	Allogeneic	No	Oncology	Chronic Graft vs Host Disease after haematopoietic stem cell transplantation
Prof S.B Dunnett	Cardiff	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.	EU	Late Preclinical		Striatal neurons	Clinical grade endothelial stem cell lines	Allogeneic	No	Neurology	Huntington's Disease
Bridget Bax	St Georges University London	Clinical development of erythrocyte encapsulated thymidine phosphorylase - a therapy for mitochondrial neurogastrointestinal encephalomyopathy.	The project will be conducted in three phases: 1) Validation of processes required for meeting regulatory requirements for operating a clinical trial project; 2) Conducting a multi-centre (pan European), open-label, multiple ascending dose, Phase II trial in 10 patients with mitochondrial neurogastrointestinal encephalomyopathy, over 36 months; and 3) Data analysis and assembly of documentation for regulatory submission.	MRC	Late Preclinical	End of 2016	Red blood cells	Blood	Autologous	No	Neurology	Mitochondrial neurogastrointestinal encephalomyopathy

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Prof. Giulio Cossu	Manchester University	Pre-clinical development of a stem cell based gene therapy protocol for Duchenne Muscular Dystrophy.	Planning to enhance each step of transplantation (adhesion to and crossing the vessel wall, migration in the muscle ECM, differentiation and enhanced gene correction) through in vitro models Oprimised conditions will be tested in immune deficient dystrophic mice as a proof of principle for developipng a new cell mediated gene therapy, optimised protocol for thesystemic delivery of autologus, genetically corrected mesoangioblasts to Duchenne Muscular Dystrophy patients.	MRC, Biodesign; EC FP7; Duchenne Parent Project.	Late Preclinical	-	Mesangioblasts	Left Extensor Digitorum Brevis	Autologous	Yes - lentiviral transduction	Neurology	Duchenne Muscluar Dystrophy Limb Girdle Muscular Dystrophy 2D
Prof. Paolo Madeddu	Bristol University	Human pericytes for the treatment of ischemia and congenital heart disease	Pericytes harvested from veins or hearts delivered in models of limb or myocardial ischaemia and in models of congenital heart disease, with standard operating procedure transferred to clinical grade facilities.	British Heart Foundation; MRC; Jules Thorn Trust.	Late Preclinical	-	Pericytes	Vein and heart	Autologous	No	Cardiovascular	Cardiac repair