Cell and Gene Therapy Catapult UK preclinical database 2016

Name	Organisation	Project title	Project summary	Funding source(s)	Stage of development	Expected completion date	Cell type	Cell source	Autologous/allogeneic	Gene modification/gene therapy	Disease area	Clinical indication	Approval to publish	If exceptions
Che Connon	Newcastle University	Scaling up of ambient cell storage using hydrogel encapsulation	Building upon previous MRC data to scale up encapsulation of stem cells for storage/transport at room temperature in collaboration with the bioprocessing community.	BBSRC, EPSRC	Early preclinical	January 2017	Mesenchymal stem / stromal cells	Commercial	Allogeneic	No	Other	All diseases	Yes	
Che Connon	Newcastle University	Battlefield cell based therapy: Can stem cells be used on the frontline?	Combined storage and delivery device for therapeutic stem cells. Cells stored for 1 month at ambient temp (i.e. in back-pack) then delivered to superficial wound site by soldier in war zone	DSTL	Early preclinical	October 2016	Other	Adipose (commercial)	Allogeneic	No	Other	Wound healing, laceration, burn etc	Yes	
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 TCRs cloned from CD8+ or CD4+ virus-specific T cell effectors.		Early preclinical		Retinal cells	Peripheral blood	Autologous	Yes, TCR gene transfer - pMP71-PRE retrovirus	Oncology	EBV+ tumours	Yes	
Jenny Southgate	University of York	Development of a novel approach for bladder augmentation utilising autologous urothelium attached to vascularised demucosalised bowel	Development of a novel approach for bladder augmentation utilising autologous urothelium attached to vascularised demucosalised bowel	MRC	Early preclinical		Other	Bladder	Autologous	No	Urology	Bladder exstrophy	Yes	
Julia Reichelt	Newcastle University	Gene therapy for keratin-associated inherited blistering skin diseases using epidermal stem cells	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.	Self-funded PhD	Early preclinical	Q4 2017	Corneal stem cells	Skin	Autologous	Yes, the disease causing mutant allele has been inactivated in patient cells using TALEN technology by via NHEJ inducing a frameshift at the target site leading to nonsense-mediated decay of the respective mRNA.	Dermatology	Epidermolysis bullosa simplex	Yes	
Paolo Madeddu	University of Bristol	Human pericyte progenitor cells and cardiac progenitor cells for specialized stimulation of neovascularization and cardiomyogenesis of the infarcted heart	Human pericyte progenitor cells and cardiac progenitor cells for specialized stimulation of neovascularization and cardiomyogenesis of the infarcted heart	MRC	Early preclinical		Human pericyte progenitor cells and cardiac progenitor cells	Vein and heart	Allogeneic	No	Cardiovascular	Cardiac repair	Yes	
Paolo Madeddu	University of Bristol	Neonatal cardiac pericytes engineered grafts for correction of congenital heart defects	Neonatal cardiac pericytes engineered grafts for correction of congenital heart defects	MRC	Early preclinical		Induced pluripotent stem cells	Vein and heart	Allogeneic	No	Cardiovascular	Cardiac repair	Yes	
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		Induced pluripotent stem cells		Both		Ophthalmology	Blindness caused by age related degeneration of retina or inherited retinal disorders	Yes	
Majlinda Lako	Newcastle University	iPS-based disease model for AMD	Assessing the feasibility of induced pluripotent stem cells to provide a disease model for age-related macular degeneration		Early preclinical		Induced pluripotent stem cells		Both		Ophthalmology	Blindness caused by age related degeneration of retina	Yes	
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 (iPSC)		Early preclinical		Induced pluripotent stem cells		Autologous		Ophthalmology	Blindness caused by inherited retinal disorders	Yes	
Majlinda Lako	Newcastle University	Stem cells for biological assays of novel drugs and predictive toxicology	This is an IMI funded project aiming at deriving human iPSC lines from 500 patients with neurodegenerative disorders. I am co-leading WP3 with Dr Lyle Armstrong	EU	Early preclinical	2017	Other		N/A		Neurology	Neurodegeneration	Yes	
Asis Palazon	University of Cambridge / Eonia therapeutics	Small molecules that promote T cell memory formation for cell therapy	It is well recognised that the clinical response of T-cell therapies depends on the ability of therapeutic T-cells to persist in treated patients, as those undergoing complete remission have robust long term persistence of these T-cells. Through our research at the University of Cambridge, we have discovered a novel pathway in T-cells that controls their ability to persist in vivo. Moreover, we have developed chemical modulators that allow us to harness this pathway for therapeutic potential. Our aim is to use this technology to generate T-cells with enhanced persistence and hence therapeutic efficacy. We also plan to generate a wider portfolio of small molecule modulators of our pathway, to further innovate and consolidate our position as a leader in this field.	Wellcome Trust	Early preclinical	Q4 2016	T-cells	Peripheral blood	Autologous	Yes, retroviral or leniviral expression	Oncology		Yes	
Paolo Madeddu	University of Bristol	Preclinical trial with human pericyte progenitors in a large animal model of myocardial infarction	Preclinical trial with human pericyte progenitors in a large animal model of myocardial infarction	MRC	Early preclinical	Q4 2016	Mesenchymal stem cells	Vein	Allogeneic	No	Cardiovascular	Cardiac repair	Yes	
Peter Jones	King's College London	Using mesenchymal stem cells to improve islet transplantation outcome	Co-culturing and co-transplanting islets with MSCs to improve survival and function of islet grafts as a treatment for type 1 diabetes	Diabetes UK	Early preclinical	2017	Pancreatic islets	Adipose, bone marrow, pancreatic	Allogeneic	No	Diabetes	Type 1 diabetes	Yes	
Paolo Madeddu	University of Bristol	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.	British Heart Foundation	Early preclinical		Pericytes	Vein and heart	Allogeneic	No	Cardiovascular	Cardiac repair	Yes	

Cell and Gene Therapy Catapult UK preclinical database 2016

Name	Organisation	Project title	Project summary	Funding source(s)	Stage of development	Expected completion date	Cell type	Cell source	Autologous/allogeneic	Gene modification/gene therapy	Disease area	Clinical indication	Approval to publish	If exceptions
Paolo Madeddu	University of Bristol	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.	MRC	Early preclinical		Bone marrow-derived cells	Bone marrow	Autologous	No	Cardiovascular	Cardiac repair (post acute myocardial infarction)	Yes	
Steve Bloor	Videregen Ltd	Tissue engineered autologous stem cell seeded liver replacement	Development of a tissue engineered liver replacement using a decellularised porcine liver, seeded with autologous cells. For the treatment of liver diseases.	Private investment / VC	Early preclinical	Q1 2017	Other	Adipose, liver, blood	Autologous	No	Hepatology	End stage liver disease e.g. cirrhosis	Yes	
Yen Choo	Plasticell Ltd	Cord blood heamatopoietic stem cells	Adult hematopoeitic stem cells expanded ex vivo	Innovate UK	Early preclinical	December 2016	CD34 and/or CD133 stem cells	Cord blood	Allogeneic	No	Oncology	Re-population of immune system following chemo/radio therapy	Yes	
Stefano Pluchino	University of Cambridge	iNSC's for progressive MS	Development of patient-specific induced neural stem cells as a therapeutic for progressive MS	Private investmen/VC, MS Societies	Early preclinical	Q4 2016	Muscle progenitor cells / epithelial progenitor cells	Skin (or other accessible sources)	Autologous	Yes, sendai virus-mediated delivery of transcription factors	Neurology	MS, stroke, spinal cord injury, traumatic brain injury	Yes	
Cedric Ghevaert	University of Cambridge	Platelets derived from iPS	iPSC derived megakaryocytes now in preclinical studies for platelet production.	MRC, NIHR, Wellcome Trust, BHF	Early preclinical	2017	Mesenchymal stem / stromal cells	Induced pluripotent stem cells (iPSCs)	Allogeneic	Yes, Combination of lentiviral transduction and genome editing with CRIPRs_ongoin non-integrative approaches	Haematology	Blood (platelets) for transfusion	Yes	
Andrew H Baker	University of Glasgow	Clinical transplantation of endothelial cells derived from human embryonic stem cells	Human embryonic stem (hES) cells and induced pluripotent stem (iPS) cells hold broad potential in regenerative medicine. Such stem cells can generate all cell types upon stimulation to differentiate along defined cell commitment pathways. We are interested in the mechanisms that govern stimulation of these stem cells into vascular ECs and their subsequent application to regenerative medicine. This present project builds upon pilot data showing that we can generate human endothelial cells from hES cell lines under GMP-compliant conditions (achieved through TSB funding). We are now developing this translational agenda towards a first-in-man trial through careful assessment of these cells. This will be achieved by final protocol optimisation and validation, safety studies, efficacy and biodistribution studies.	MRC	Mid preclinical	Q4 2016	Human amniotic fluid stem cells	Human embryonic stem cells	Allogeneic	No	Cardiovascular	Peripheral limb ischemia	Yes	
Steve Lee	University of Birmingham	Engineering human T cells to target the tumour vasculature through expression of a chimeric antigen receptor	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.		Mid preclinical	Q3 2016	T-cells	Peripheral blood	Autologous	Yes, CAR gene transfer	Oncology	Multiple common solid tumours	Yes	
G Astrid Limb	University College London, Institute of Ophthalmology	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.	MRC	Mid preclinical		Adult derived cardiac stem cells	Retina	Allogeneic	No	Ophthalmology	Glaucoma, retinitis pigmentosa and age-related macular degeneration (AMD)	Yes	
Marc Turner	Scottish National Blood Transfusion Service (SNBTS)	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.	Wellcome Trust, Scottish Funding Council	Mid preclinical	2016	T-cells	Human induced pluripotent stem cells (hiPSCs)	Allogeneic	No	Haematology	Beta thalassaemia	Yes	
Steve Bloor	Videregen Ltd	Tissue engineered autologous stem cell seeded bowel replacement	Develop a tissue engineered bowel for treatment of short bowel syndrome. Utilises decellularisation/recellularisation technology .	Private investment/VC, IKC medical technologies grant	Mid preclinical	Q1 2017	Mesenchymal stem / stromal cells	Bowel and adipose tissue	Autologous	No	Gastroenterology	Short bowel syndrome (Crohn's' disease, necrotising enterocolitis)	Yes	
Sue Kimber	University of Manchester	Pluripotent stem cell-derived cartilage cells	Pluripotent stem cell-derived cartilage cells	MRC, ARUK, EU FP7	Mid preclinical		Mesenchymal stem / stromal cells		Allogeneic	No	Orthopaedics	Osteoarthritis, sports injury and similar conditions	Yes	
Anna David	University College London	EVERREST	Does vascular endothelial growth factor gene therapy safely improve outcome in severe early-onset fetal growth restriction? Adenovirus VEGF Gene therapy will be given to women with a diagnosis of severe early onset fetal growth restriction (22-26+6 weeks of gestation, estimated fetal weight <3rd centile and not growing).	Magnus growth capital investment, EU FP7	Late preclinical	Q4 2018	Fetal liver mesenchymal stem cells	N/A	N/A	N/A	Obstetrics	Fetal growth restriction	Yes	see www.everrest-fp7.eu
Anna David	University College London	BOOSTB4	Boost Brittle Bones Before Birth. A clinical trial to compare in utero plus postnatal mesenchymal stem cell transplantation with postnatal transplantation alone, in fetuses with a molecular diagnosis of severe osteogenesis imperfecta. Phase I trial of safety and comparing with historical controls for efficacy. To be conducted in 4 EU centres of excellence, UCL/GOSH London, Leiden Netherlands, Koln Germany, Karolinska Sweden	EU Horizon 2020	Late preclinical	Q4 2016	T-cells	First trimester terminations of pregnancy at Karolinska Tnstitutet, Stockholm	Allogeneic	No	Congenital skeletal dysplasia	Severe osteogenesis imperfecta	Yes	see http://cordis.europa.eu/project/rcn/198792_en.html

Cell and Gene Therapy Catapult UK preclinical database 2016

Name	Organisation	Project title	Project summary	Funding source(s)	Stage of development	Expected completion date	Cell type	Cell source	Autologous/allogeneic	Gene modification/gene therapy	Disease area	Clinical indication	Approval to publish	If exceptions
John Campbell	Scottish National Blood Transfusion Service (SNBTS)	Endothelial outgrowth cells for blood vessel repair	Prof. David Newby and Prof. Nick Mills at University of Edinburgh have identified endothelial progenitor cells in blood that can be cultured as a potential therapeutic for blood vessel repair	BHF	Late preclinical	2016	Lymphocytes	Peripheral blood	Autologous	No	Vascular	Blood vessel repair	Yes	
John Campbell	Scottish National Blood Transfusion Service (SNBTS)	Virus-Specific T cells	SNBTS and Universities of Edinburgh and Aberdeen have established 2 banks of HLA-typed, EBV-specific T cells to treat EBV-drive post transplant lymphoproliferative disease (PTLD). These products have been used to treat approx. 100 patients and has successfully completed a phase 2 clinical trial. The current project aims to extensively re-develop the T cell generation process, in order to remove live virus, non-human culture components, and to significantly shorten the time taken to generate T cell lines.	SNBTS	Late preclinical	2017	Other	Peripheral blood	Allogeneic	No	Vascular	Virally-driven diseases including tumours	Yes	
Prof Madrigal and Dr Saudemont	Anthony Nolan 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	December 2016	Other	Umbilical cord blood	Allogeneic	No	Oncology	Chronic Graft versus Host Disease after haematopoietic stem cell transplantation	Yes	
Giulio Cossu	University of Manchester	Clinical proof of principle for Duchenne Muscular Dystrophy	In order to move from donor cell transplantation (that requires immune suppression) to autologous genetcially corrected cells, we plan a “proof of principle” stem cell mediated gene therapy trial, based on local, intra-muscular transplantation of autologous, genetically corrected Mabs, in DMD. The trial "Mesoangioblast-mediated exon skipping for genetic correction of exon 51 mutation, based upon a single injection in individual skeletal muscles of five non ambulant patients affected by Duchenne Muscular Dystrophy: a non randomized, open label, phase I/IIa study" is planned to start in 2016.	Wellcome Trust	Late preclinical		Other	Left extensor digitorum brevis	Autologous	Yes, lentiviral transduction	Neurology		Yes	
Giulio Cossu	University of Manchester	Preclinical development of a stem cell based gene therapy protocol for Duchenne Muscular Dystrophy	We recently completed a “first in man” phase I/IIa clinical trial based upon intra-arterial transplantation of HLA-matched, donor mesoangioblasts (vessel associated myogenic progenitors) in five patients affected by Duchenne Muscular Dystrophy (DMD). Overall the trial was safe but showed minimal efficacy. In order to approach efficacy and plan the future development of this strategy we are 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 DMD patients. .	Wellcome Trust, Biodesign, EC FP7 Marató (Spanish Fundation) Duchenne Parent Project	Late preclinical		Other	Left extensor digitorum brevis	Autologous	Yes, Lentiviral transduction	Neurology	Duchenne muscluar dystrophy limb girdle muscular dystrophy 2D	Yes	
Paolo Madeddu	University of Bristol	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.	MRC, British Heart Foundation, Jules Thorn Foundation	Late preclinical	Q4 2016	Mesenchymal stem / stromal cells	Vein and heart	Autologous	No	Cardiovascular	Cardiac repair	Yes	
Sam Janes	University College London	MSC-TRAIL	MSCs genetically engineered to express TNF related apoptosis ligand (TRAIL) as a treatment for lung cancer.	MRC	Late preclinical	31st March 2017	Neural stem cells	Mesenchymal stem cells	Allogeneic	Yes, engineered to express TNF related apoptosis ligand (TRAIL) - lentivirus	Oncology	Non-small cell lung cancer (adenocarcinoma)	Yes	
Stephen Dunnett	Cardiff University	Repair-Huntington's Disease	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	2017	Other	Clinical grade endothelial stem cell lines (Roslin RC9 ...) and newly generated fetal-derived iPS cells (sourced from CFTB)	Allogeneic		Neurology	Neurological (Huntington's disease)	Yes	n/a
Steve Bloor	Videregen Ltd	Tissue engineered autologous cell seeded mucosal lining replacement	Development of a tissue engineered epithelial/ airway mucosal replacement using a decellularised mucosal scaffold, seeded with autologous derived epithelial cells. For the treatment of airway mucosal lining following mucosal lining injury e.g. truma, cancer.	Private investment/VC, Innovate UK	Late preclinical	Q1 2017	Mesenchymal stem / stromal cells	Autologous airwaY mucosal	Autologous	No	Respiratory medicine	Airway disease, injury	Yes	
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 MSCs and airway epithelial cells. For the treatment of severe structural airway diseases.	Innovate UK, Private investment/VC, EU	Late preclinical	Q1 2016		Bone marrow and airway	Autologous	No	Respiratory medicine	Structural airway diseases	Yes	

Cell and Gene Therapy Catapult UK preclinical database 2016

Name	Organisation	Project title	Project summary	Funding source(s)	Stage of development	Expected completion date	Cell type	Cell source	Autologous/allogeneic	Gene modification/gene therapy	Disease area	Clinical indication	Approval to publish	If exceptions
Marcelo Rivolta	University of Sheffield	Otic neuroprogenitors for deafness	hESC derived otic neuroprogenitors for deafness, examination of function in animal models										Yes, but with exceptions	Only the first two questions 'About you' and 'About the project' can be published. The rest I would like to remain confidential