Cell and Gene Therapy Catapult clinical trials database 2018

Name of Sponsor	Title	Project Summary	Clinical Database Numbers	United Kingdom Site(s)	Clinical Trial Status	Trial Phase	Year Trial Started	Recruitment Target	Cell Type	Gene Modification/ Gene Therapy	If applicable, type of virus vector used	Autologous/ Allogeneic	Disease Area	Indication
Cell Medica Inc	WT1 TCR Gene Therapy for Leukaemia: A Phase I/II Safety and Toxicity Study (WT1 TCR-001)	Approximately 18 HLA-A*0201 positive subjects with either AML not suitable for BMT or at first relapse post-BMT or CML in chronic phase resistant to imatinib and/or second-generation tyrosine kinase inhibitors and not eligible for BMT will be recruited. The safety, tolerability and efficacy of the WT1 TCR-td T cells will be determined through clinical evaluation and assessment of T cell engraftment kinetics and immunological and functional potential of TCR-td autologous T cells.	2006-004950-25 NCT01621724	University College London Hospitals NHS Trust, London University Hospitals Bristol NHS Foundation Trust, Bristol	5- In follow-up	Phase I/II	2012	18	T cells	Yes ex-vivo	Retrovirus	Autologous	Cancer (Haematology)	AML CML
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	2007-000684-16	Great Ormond Street Hospital, London	5- In follow-up	Phase I/II	2011	10	CD34 and/or CD133 stem cells	Yes ex-vivo	Retrovirus	Autologous	Inflammatory and immune system	X-linked severe combined immunodeficiency
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 autologus 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	2010-024253-36; NCT01380990	Great Ormond Street Hospital, London	5- In follow-up	Phase I/II	2012	10	CD34 and/or CD133 stem cells	Yes ex-vivo	Lentivirus	Autologous	Inflammatory and immune system	Adenosine Deaminase Deficiency
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	London Chest Hospital, Barts and The London NHS Trust, London The Heart Hosptial, UCLH Foundation Trust, London The Royal Free Hospital, Royal Free London Foundation Trust, London	5- In follow-up	Phase II	2007	100	Bone marrow mononuclear cells	No		Autologous	Cardiovascular	Acute myocardial infarction
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	New Cross Hospital, Wolverhampton Barts Health NHS trust, London	5- In follow-up	Phase III	2011	350-400	Bone marrow mononuclear cells	No		Autologous	Cardiovascular	Acute myocardial infarction
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	Cardiff University Imperial College London University College London University of Cambridge	5- In follow-up	Phase I/II	2012	40: 20 transplanted patients, 20 controls	Neural	No		Allogeneic	Neurological	Parkinson's disease
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 ClinTrials: NCT01151124	Glasgow Southern General Hospital	5- In follow-up	Phase I	2010	12	Neural	Yes ex-vivo	Retrovirus	Allogeneic	Neurological	Ischaemic stroke
ReNeuron Limited, UK	A Phase II 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 ClinTrials: NCT02117635	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.	5- In follow-up	Phase II	2014	21	Neural	Yes ex-vivo	Retrovirus	Allogeneic	Neurological	Ischaemic stroke
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 ClinTrials: NCT01916369	Ninewells Hospital, Dundee	5- In follow-up	Phase I	2014	9	Neural	Yes ex-vivo	Retrovirus	Allogeneic	Cardiovascular	Peripheral Arterial Disease- lower limb ischaemia
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)	2011-000608-16 51772481 UK CRN 11185	Newcastle RVI	5- In follow-up	Phase II	2012	24	Corneal	No		Autologous	Eye	Limbal stem cell deficiency
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 expandedstem 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.	2010-022072-31	The Robert Jones and Agnes Hunt Orthopaedic Hospital NHS Foundation Trust	3- Recruiting	Phase II	2013	114	Mesenchymal stem/stromal cells	No		Autologous	Bone and cartilage	Osteochondral defects of the knee (early osteoarthritis)
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	2010-024162-22		3- Recruiting	Phase I/II	2012	10	Mesenchymal stem/stromal cells	No		Autologous	Bone and cartilage	Knee meniscus repair
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. The main study has completed but we are now recruiting to a small extension study, which is identical except the ttoIDC are lablled with indium-111 to enable us to track them in vivo using SPECT-CT up to 72 hours from administration.	NCT01352858 87426082 UK CRN 12108	Newcastle RVI	3- Recruiting	Phase I	2011	12 plus 3 in extension study	Antigen presenting cells	No		Autologous	Inflammatory and immune system	Rheumatoid and Inflammatory Arthritis
University College London	CMV TCR Gene Therapy: A Phase I Safety, Toxicity and Feasibility Study of Adoptive Immunotherapy with CMV TCR-transduced Donor-derived T cells for Recipients of Allogeneic Haematopoietic Stem Cell Transplantation	CMV TCR Gene Therapy: A Phase I Safety, Toxicity and Feasibility Study of Adoptive Immunotherapy with CMV TCR-transduced Donor-derived T cells for Recipients of Allogeneic Haematopoietic Stem Cell Transplantation	UK CRN 12518 2008-006649-18	University College London Hospital	3- Recruiting	Phase I	2013	10	T cells	Yes ex-vivo	Retrovirus	Allogeneic	Cancer (Haematology)	CMV seronegative HSCT donors & CMV seropositive HSCT recipients
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	Manchester Royal Infirmary, University College London Hospital, London	3- Recruiting	Phase II	2014	24	T cells	No		Allogeneic	Cancer (Haematology)	Haematological malignancies
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 Hospital, London	3- Recruiting	Phase I	2015	30	T cells	Yes ex-vivo	Retrovirus	Autologous	Cancer	Head and neck cancer
Cell Medica Inc	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 (CITADEL Study)	NCT01948180	University College London Hospital, London The Christie Clinic, Manchester	5- In follow-up	Phase II	2015	35	T cells	No		Autologous	Cancer (Haematology)	NK/T cell lymphoma
Cell Medica Inc	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) who have failed to achieve or maintain an IWG defined response following hypomethylating agent therapy.	Approximately 25 HLA-A*0201 positive subjects with either Intermediate Risk-2 or High-Risk MDS, or AML with less than 30% bone marrow blasts, who have failed to achieve or maintain an IWG response following hypomethylating agent therapy (at least 6 cycles of azacitidine or 4 cycles of decitabine) will be recruited. The safety, tolerability and efficacy of the WT1 TCR-td T cells will be determined through clinical evaluation and assessment of T cell engraftment kinetics and immunological and functional potential of TCR-td autologous T cells.	2014-003111-10 NCT02550535	University College London Hospitals NHS Trust, London University Hospitals Bristol NHS Foundation Trust, Bristol Leeds Teaching Hospitals NHS Trust, Leeds University Hospital of Wales, Cardiff	5- In follow-up	Phase I/II	2015	25	T cells	Yes ex-vivo	Retrovirus	Autologous	Cancer (Haematology)	MDS AML
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	2015-001586-96	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.	5- In follow-up	Phase I/II	2015	40	Mesenchymal stem/stromal cells	No		Allogeneic	Respiratory	Acute Respiratory Distress Syndrome

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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	Guy's and St Thomas' NHS Foundation Trust	5- In follow-up	Phase I	2015	5	Fibroblasts	Yes ex-vivo	Lentivirus	Autologous	Skin	Recessive dystrophic epidermolysis bullosa
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 London	3- Recruiting	Phase I/II	2012	50	Skeletal Muscle	No		Autologous	Musculoskeletal	Faecal Incontinence
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	3- Recruiting	Phase II	2015	10	Mesenchymal stem/stromal cells	No		Autologous	Musculoskeletal	Achilles Tendinopathy
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, London	3- Recruiting	Phase I	2015	12	T cells	Yes ex-vivo	Lentivirus	Autologous	Cancer (Haematology)	Diffuse Large B-Cell Lymphoma
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.	NCT02443831	Great Ormond Street Hospital for Children London, United Kingdom, WC1N 3JH University College London Hospital London, United Kingdom; Royal Manchester Children's Hospital	3- Recruiting	Phase I/II	2016	18	T cells	Yes ex-vivo	Lentivirus	Autologous	Cancer (Haematology)	Paediatric Acute Lymphoblastic Leukaemia and other haematological malignancies (e.g. Burkitt's lymphoma)
The University of Edinburgh	Macrophage Therapy for Liver Cirrhosis (MATCH)	A single, phase I/II trial of repeated infusions of autologous CD14+ monocyte-derived macrophages in patients with liver cirrhosis	2015-000963-15	Edinburgh Royal Infirmary	3- Recruiting	Phase I/II	2016	63	Other	No		Autologous	Liver	Advanced Liver Cirrhosis
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 EudraCT: 2016-000661-23	Belfast Health and Social Care Trust - Belfast City Hospital Belfast, United Kingdom University Hospital Birmingham NHS Foundation Trust - Queen Elizabeth Medical Centre Birmingham, United Kingdom	2- In set-up	Phase I/II	2017	48	Mesenchymal stem/stromal cells	No		Allogeneic	Renal and Urogenital	Diabetic kidney disease
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	Heartlands Hospital Not yet recruiting Birmingham, United Kingdom, B9 5SS Hammersmith Hospital Recruiting London, United Kingdom, W12 ONN	3- Recruiting	Phase II	2015	15	T cells	No		Allogeneic	Cancer (Haematology)	Acute Myeloid Leukaemia (AML), Acute Lymphoblastic Leukaemia (ALL) and Myelodysplastic Syndrome (MDS)
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.	3- Recruiting	Phase I/II	2016	44	CD34 and/or CD133 stem cells	No		Autologous	Cardiovascular	Acute Myocardial Infarction
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 Recruiting London, United Kingdom, WC1N 1EH Royal Free Hospital Recruiting London, United Kingdom, WC1N 1EH	3- Recruiting	Phase I/II	2011	5	CD34 and/or CD133 stem cells	Yes ex-vivo	Lentivirus	Autologous	Inflammatory and immune system	Wiskott-Aldrich Syndrome (WAS)
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 for Children NHS Trust London,	5- In follow-up	Phase I/II	2011	1	CD34 and/or CD133 stem cells	Yes ex-vivo	Retrovirus	Autologous	Inflammatory and immune system	X-linked Severe Combined Immunodeficiency
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	University College London Hospital (UCLH) Recruiting London, United Kingdom, NW1 2PG Royal Free Hospital (RFH) Recruiting London, United Kingdom, NW3 2QG Great Ormond Street Hospital NHS Foundation Trust London, United Kingdom	3- Recruiting	Phase I/II	2013	5	CD34 and/or CD133 stem cells	Yes ex-vivo	Lentiviral vector	Autologous	Inflammatory and immune system	X-Linked Chronic Granulomatous Disease (X-CGD)
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	3- Recruiting	Phase I	2014	180	T cells	Yes ex-vivo	Retrovirus	Allogeneic	Blood	Hematological disorders
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)	2011-005798-22 NCT01656902	Royal Devon and Exeter Hospital Exeter, United Kingdom, EX2 5DW	3- Recruiting	Phase III	2012	261	Chondrocytes	No		Autologous	Bone and cartilage	Articular cartilage defects of the knee
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	3- Recruiting	Phase I	2016	10	T cells	Yes ex-vivo	Lentivirus	Allogeneic	Cancer (Haematology)	B-cell acute lymphoblastic leukemia
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			2- In set-up	Phase II	2016	12	Other	No		Autologous	Metabolic and Endocrine	Mitochondrial neurogastrointestinal encephalomyopathy (MNGIE)
Astellas Institute for Regenerative Medicine	Follow-up to 5 Years of 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)	The purpose of this study is to evaluate the safety and tolerability of hESC-RPE cellular therapy in patients with advanced SMD over a five-year period following the surgical procedure to implant the cells.This study is a long-term, extension of a Phase I/II, open-label, non-randomized, 4-cohort, multi-center clinical trial (referred to as the core trial or core protocol) in which a maximum of 12 SMD patients were transplanted with sequential doses of hESC-RPE cells, starting at a dose of 50,000 hESC-RPE cells transplanted and increasing to a maximum dose of 200,000 hESC-RPE cells transplanted.	NCT02941991	Moorefields Eye Hospital NHS Foundation Trust, London, United Kingdom, EC1V2PD Newcastle on Tyne NHS Foundation Trust Newcastle upon Tyne, United Kingdom, NE7 7DN	5- In follow-up	Phase I/II	2013	11	Retinal	No		Allogeneic	Eye	Stargardt's Macular Dystrophy

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Cynata Therapeutics Limited	An Open-Label Phase 1 Study to Investigate the Safety and Efficacy of CYP-001 for the Treatment of Adults With Steroid-Resistant Acute Graft Versus Host Disease	The purpose of this study is to assess the safety, tolerability and efficacy of two infusions of CYP-001 in adults with steroid-resistant GvHD. This is a multi-centre, open label, dose escalation study to assess the safety, tolerability and efficacy of two infusions of CYP-001, in adults who have steroid-resistant GvHD. Participants will receive standard of care treatment throughout the study, according to local procedures. The first eight participants will be enrolled in Cohort A and receive a CYP-001 dose of 1 million cells per kg, up to a maximum dose of 100 million cells, on Day 0 and Day 7. Subject to a safety review of data from Cohort A, an additional eight participants will be enrolled into Cohort B and receive a CYP-001 dose of 2 million cells/kg, up to a maximum dose of 200 million cells, on Day 0 and Day 7. The primary evaluation period concludes for each participant 100 days after the first dose of CYP-001. Participants will have study visits on Days 0, 3, 7, 14, 21, 28, 60 and 100. Subsequently, participants will enter a long term follow-up period, which concludes 2 years after the first dose of CYP-001.	NCT02923375	NHS Foundation Trust Recruiting Manchester, United Kingdom	5- In follow-up	Phase I	2016	16	Mesenchymal stem/stromal cells	Yes ex-vivo	iPSC derivation	Allogeneic	Inflammatory and immune system	Steroid-Resistant Acute Graft Versus Host Disease
Belfast Health and Social Care Trust	Repair of Acute Respiratory Distress Syndrome by Stromal Cell Administration (REALIST): An Open Label Dose Escalation Phase 1 Trial Followed by a Randomized, Double-blind, Placebo-controlled Phase 2 Trial	Acute Respiratory Distress Syndrome (ARDS) causes the lungs to fail due to the collection of fluid in the lungs (pulmonary oedema). ARDS is common in severely ill patients in Intensive Care Units and is associated with a high mortality and a high morbidity in those who survive. There is a large economic burden with direct healthcare costs, but also indirectly due to the impact on the carer and patient through the patients inability to return to full time employment. There is little evidence for effective drug (pharmacological) treatment for ARDS. There is increasing information that mesenchymal stem cells (MSCs) might be important in treating ARDS. REALIST will investigate if a single infusion of MSCs will help in the treatment of ARDS. The first step will be to first of all determine what dose of MSCs is safe and then divide patients suffering from ARDS into two groups, one of which will get MSCs and the other a harmless dummy (or placebo) infusion, who will then be followed up to determine if lung function improves. If effective this may lead to further research to determine if MSCs are effective in patients with ARDS. This project will also provide new information about mechanisms in the development of ARDS leading, potentially, to other new treatments	NCT03042143 Eudract 2017-000584-33	Belfast Health and Social Care Trust, Royal Hospitals	2- In set-up	Phase I/II	2017	84	Mesenchymal stem/stromal cells	No		Allogeneic	Respiratory	Acute Respiratory Distress Syndrome
Bluebird Bio	A Phase 3 Single Arm Study Evaluating the Efficacy and Safety of Gene Therapy in Subjects With Transfusion-dependent β-Thalassemia, Who do Not Have β0/β0 Genotype, by Transplantation of Autologous CD34+ Stem Cells Transduced Ex Vivo With a Lentiviral βA-T87Q-Globin Vector in Subjects ≥12 and ≤50 Years of Age	This is a single-arm, multi-site, single-dose, Phase 3 study in approximately 15 subjects ≥12 and ≤50 years of age with transfusion-dependent β-thalassemia (TDT), also known as β-thalassemia major, who do not have a β0 mutation at both alleles of the hemoglobin β (HBB) gene. The study will evaluate the efficacy and safety of autologous hematopoietic stem cell transplantation (HSCT) using LentiGlobin BB305 Drug Product.	NCT02906202	London	2- In set-up	Phase III	2016	15	CD34 and/or CD133 stem cells	Yes ex-vivo	Lentivirus	Autologous	Blood	Transfusion-Dependent β-Thalassemia
Institut de Recherches Internationales Servier, France	Phase I, Open Label, Dose-escalation Study to Evaluate the Safety, Expansion, Persistence and Biological Activity of a Single Dose of UCART19 (Allogeneic Engineered T-cells Expressing Anti-CD19 Chimeric Antigen Receptor), Administered Intravenously in Patients With Relapsed or Refractory CD19 Positive B-cell Acute Lymphoblastic Leukaemia (B-ALL) or Chronic Lymphocytic Leukaemia (CLL)	The purpose of this study is to evaluate the safety and tolerability of several doses of UCART19 in patient with relapsed / refractory (R/R) acute lymphoblastic leukaemia (ALL) or chronic lymphocytic leukaemia (CLL)	NCT02746952	King's College Hospital NHS Foundation Trust Christie NHS Foundation Trust	3- Recruiting	Phase I	2016	11	T cells	Yes ex-vivo	Lentivirus	Allogeneic	Cancer (Haematology)	B-cell acute lymphoblastic leukemia
Iovance Biotherapeutics, Inc.	Study of LN-145, Autologous Tumor Infiltrating Lymphocytes in the Treatment of Patients With Cervical Carcinoma	A Phase 2, Multicenter Study to Evaluate the Efficacy and Safety Using Autologous Tumor Infiltrating Lymphocytes (LN-145) in Patients With Recurrent, Metastatic or Persistent Cervical Carcinoma	2016-003447-11	Iovance Cervical Investigative Site	3- Recruiting	Phase II	2017	47	T cells	No		Autologous	Cancer	Cervical Carcinoma
Kiadis Pharma	A Phase III, Multicenter, Randomized Controlled Study to Compare Safety and Efficacy of a Haploidentical HSCT and Adjunctive Treatment With ATIR101, a T-lymphocyte Enriched Leukocyte Preparation Depleted ex Vivo of Host Alloreactive T-cells, Versus a Haploidentical HSCT With Post-transplant Cyclophosphamide in Patients With a Hematologic Malignancy	The primary objective of this study is to compare safety and efficacy of a haploidentical T-cell depleted HSCT and adjunctive treatment with ATIR101 versus a haploidentical T cell replete HSCT with post-transplant administration of high dose cyclophosphamide (PTCy) in patients with a hematologic malignancy. An additional objective of the study is to compare the effect of the two treatments on quality of life.	2016-004672-21	Heartlands Hospital. St James University Hospital. Royal Liverpool University Hospital. Hammersmith Hospital. Manchester Royal Infirmary.	3- Recruiting	Phase III	2017	195	T cells	No		Allogeneic	Cancer (Haematology)	AML ALL MS
Celgene Corp	A Study to Compare the Efficacy and Safety of JCAR017 to Standard of Care in Adult Subjects With High-risk, Transplant-eligible Relapsed or Refractory Aggressive B-cell Non-Hodgkin Lymphomas (TRANSFORM)	This is a randomized, open-label, parallel-group, multi-center trial in adult subjects with Relapsed or refractory (R/R) aggressive Non-Hodgkin lymphoma (NHL) to compare safety and efficacy between the standard of care (SOC) strategy versus JCAR017 (also known as lisocabtagene maraleucel or liso-cel). Subjects will be randomized to either receive SOC (Arm A) or to receive JCAR017 (Arm B).	NCT03575351	UCL Cancer Institute London, United Kingdom, WC1E 6BT University Hospital Southampton NHS Foundation Trust - Southampton General Hospital☐ Southampton, United Kingdom, SO16 6YD	3- Recruiting	Phase III	2018	182	T cells	Yes ex-vivo	Lentivirus	Autologous	Cancer (Haematology)	B-Cell Non-Hodgkin Lymphoma
Amphera BV	DENdritic Cell Immunotherapy for Mesothelioma (DENIM)	This study is to evaluate the overall survival (OS) rate (determined from the time of randomization in the study) of subjects who receive dendritic cell immunotherapy with MesoPher plus best supportive care (BSC) compared to BSC alone.	NCT03610360	University of Leicester☐ Leicester, United Kingdom	3- Recruiting	Phase II/III	2018	230	Antigen presenting cells	No		Autologous	Cancer	Malignant Mesothelioma
Celgene Corp	Trial to Determine the Efficacy and Safety of JCAR017 in Adult Subjects With Aggressive B-Cell Non-Hodgkin Lymphoma	Phase 2 single-arm, multi-cohort study to determine the efficacy and safety of JCAR017 (autologous T cells expressing anti-CD19 chimeric antigen receptor) in adult subjects with aggressive B-NHL (diffuse large B-cell lymphoma (DLBCL) NOS [de novo or transformed follicular lymphoma (tFL)], double/triple-hit lymphoma [DHL/THL], follicular lymphoma Grade 3B [FL3B], primary central nervous system lymphoma [PCNSL] and Richter's transformation).	NCT03484702	UCL Cancer Institute☐ London, United Kingdom, WC1E 6BT The Christie NHS Foundation Tru☐ Manchester, United Kingdom, M20 4BX	3- Recruiting	Phase II	2018	124	T cells	Yes ex-vivo	Lentivirus	Autologous	Cancer (Haematology)	Non-Hodgkin Lymphoma
Autolus Limited	Phase I/II Study Evaluating AUTO4 in Patients With TRBC1 Positive T Cell Lymphoma	The purpose of this study is to test the safety and efficacy of AUTO4 a CAR T cell treatment targeting TRBC1 in patients with relapsed or refractory TRBC1 positive selected T-Non-Hodgkin Lymphoma.	NCT03590574	University College London Hospitals NHS Foundation Trust The Christie NHS Foundation Trust☒anchester, United Kingdom Freeman Hospital, The Newcastle upon Tyne	3- Recruiting	Phase I/II	2018	55	T cells	Yes ex-vivo	Retrovirus	Autologous	Cancer (Haematology)	Non-Hodgkin Lymphoma
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	Addenbrookes Hospital, Cambridge	5- In follow-up	Phase I/II	2011	15		Yes in-vivo	Lentivirus		Neurological	Parkinson's Disease
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	Moorfields Eye Hospital NHS Foundation Trust, London	5- In follow-up	Phase III	2016	36		Yes in-vivo	AAV2		Eye	Leber Hereditary Optic Neuropathy (LHON)
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	Moorfields Eye Hospital NHS Foundation Trust, London	5- In follow-up	Phase III	2016	36		Yes in-vivo	AAV2		Eye	Leber Hereditary Optic Neuropathy (LHON)

Cell and Gene Therapy Catapult clinical trials database 2018

Name of Sponsor	Title	Project Summary	Clinical Database Numbers	United Kingdom Site(s)	Clinical Trial Status	Trial Phase	Year Trial Started	Recruitment Target	Cell Type	Gene Modification/ Gene Therapy	If applicable, type of virus vector used	Autologous/ Allogeneic	Disease Area	Indication
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.	3- Recruiting	Phase I/II	2015	15		Yes in-vivo	AAV		Blood	Haemophilia A
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	University Hospitals Birmingham. Cambridge University Hospital. University College London. University Hospital Wales. University of Manchester, St. Mary's Hospital.	3- Recruiting	Phase I/II	2015	44		Yes in-vivo	Single stranded antisense oligonucleotide (ASO)		Neurological	Huntington's disease
MeiraGTx UK II Ltd	An Open Label, Multi-centre, Phase I/II Dose Escalation Trial of a Recombinant Adeno-associated Virus Vector (AAV2/8-hCARp.hCNGB3) for Gene Therapy of Adults and Children With Achromatopsia Owing to Defects in CNGB3	Achromatopsia is a recessively inherited condition characterised by a lack of cone photoreceptor function resulting in impairment of colour vision and visual acuity, central scotoma often with eccentric fixation, disabling hypersensitivity to light (photophobia) and involuntary eye movements (pendular nystagmus). Children with CNGB3-related achromatopsia have profound sight impairment from birth or early infancy. The condition is currently untreatable, but there is a real possibility that a gene therapy could offer a significant benefit in terms of improved sight and quality of life (QOL), based on our own and others experience of ocular gene therapy trials and pre-clinical data demonstrating improved outcome in CNGB3- related achromatopsia. Possible benefits of improved cone-photoreceptor function include improved visual acuity; improved colour perception; and relief from disabling photophobia. Although younger individuals may benefit most from gene supplementation therapy by virtue of their greater visual plasticity, it is anticipated that the intervention may offer benefit across a range of ages and the aim is to define this range. For this reason, participants of various ages will be included; children will be included only after an acceptable safety profile has been established in adults.	NCT03001310	Moorfields Eye Hospital NHS Foundation Trust, London, UK	3- Recruiting	Phase I/II	2016	18		Yes in-vivo	AAV2		Eye	Achromatopsia
MeiraGTx UK II Ltd	An Open-label, Multi-centre, Phase I/II Dose Escalation Trial of an Adeno Associated Of an Adeno-Associated Virus Vector (AAV2/5-OPTIRPE65) for Gene Therapy of Adults And Children With Retinal Dystrophy Associated With Defects in RPE65 (LCA)	Leber congenital amaurosis (LCA) is a severe, early-onset form of inherited retinal degeneration involving both rod and cone photoreceptors. LCA is caused by mutations in one of at least 19 different genes.Mutations in RPE65, which is expressed in the retinal pigment epithelium (RPE), are responsible in 3 to 16 % of people affected. The RPE65 gene encodes a 65-kDa retinal pigment epithelium (RPE)-specific protein that is required for the conversion of vitamin A to 11-cis-retinal by the RPE and is essential for the regeneration of the rod visual pigment. Inherited defects in the gene encoding RPE65 cause night blindness and progressive loss of sight in children. There is currently no approved treatment available. In previous clinical trials we and other have shown that subretinal administration of recombinant AAV2 vectors containing cDNA for human RPE65 is generally well-tolerated and can improve low-luminance vision (night vision) in affected children and young adults (Bainbridge et al 2008; Maguire et al 2008; Cideciyan et al 2008; Bainbridge et al 2015). However, the efficacy of the intervention in humans is lower than that measured in mice and dogs with a similar condition. We hypothesise that higher efficacy can be achieved in affected humans by improving the efficiency of expression of RPE65 through optimisation of the vector construct. The purpose of this trial is to determine whether administration of a new version of the vector which has been optimised for efficiency of both transduction and RPE65 protein production, is safe and can result in greater efficacy.	NCT02781480	Moorfields Eye Hospital NHS Foundation Trust Recruiting London, United Kingdom, EC1V 2PD	3- Recruiting	Phase I/II	2016	27		Yes in-vivo	AAV2		Eye	Leber Congenital Amaurosis
MeiraGTx UK II Ltd	Long-term Follow-up Study of Participants Following an Open Label, Multi-centre, Phase I/II Dose Escalation Trial of an Adeno-associated Virus Vector (AAV2/5-OPTIRPE65) for Gene Therapy of Adults and Children With Retinal Dystrophy Owing to Defects in RPE65 (LCA2)	This study is a longer-term follow-up study for patients who have been administered AAV2/5-OPTIRPE65 in the Phase I/II, open label, non-randomised, two-centre, dose escalation trial in adults and children with retinal dystrophy associated with defects in RPE65. The study is designed to collect data on longer-term safety and efficacy up to 60-month time-points following administration.	NCT02946879	Moorfields Eye Hospital NHS Foundation Trust Recruiting London, United Kingdom	3- Recruiting	Phase I/II	2016	27		Yes in-vivo	AAV2		Eye	Leber Congenital Amaurosis
University College, London	GO-8: Gene Therapy for Haemophilia A Using a Novel Serotype 8 Capsid Pseudotyped Adeno-associated Viral Vector Encoding Factor VIII-V3	Haemophilia A is an x-linked, life threatening bleeding disorder arising from defects in the coagulation factor VIII (FVIII) gene. Current treatment for haemophilia A, the commonest inherited bleeding disorder (prevalence of 1 in 5000 individuals) consists of life-long, 2-3X/week, intravenous injection of clotting factor concentrates, which is demanding, exceedingly expensive not widely available nor curative. In contrast, gene therapy offers the potential of a cure for haemophilia A as illustrated by our first unequivocal success in a related condition, haemophilia B. In that study the investigators showed that a single intravenous administration of a serotype 8 based adeno-associated virus, (AAV8) vector encoding the factor IX (FIX) gene resulted in stable (>6 years) therapeutic expression of FIX without long-lasting toxicity. The investigators plan to use the same AAV8 platform to evaluate a novel FVIII expression cassette, AAV2/8-HLP-FVIII-V3, in patient with haemophilia A. Extensive preclinical studies demonstrate that AAV2/8-HLP-FVIII-V3 leads to long-term, endogenous expression of FVIII in mouse and non-human primate models without toxicity even when fifty-fold higher doses than the proposed starting clinical trial dose were used. Therefore, an open label, Phase I/II dose escalation study entailing a single systemic administration of AAV2/8-HLP-FVIII-V3 in adults (>18 years of age) with severe haemophilia A who have baseline factor FVIII levels of <1% of normal has been designed to establish safety and efficacy of our approach. Dosing will begin at 6x10^11 vector genome (vg)/kg progressing sequentially to 2x10^12vg/kg and ultimately 6x10^12vg/kg in the absence of toxicity. A minimum of 2 patients will be recruited at each dose with a possibility of expanding the dose cohort to a maximum of 6 patients based on safety and efficacy. The study duration for each patient will be 15 years after vector infusion.	NCT03001830	Royal Free Hospital London, United Kingdom, NW3 2QG	2- In set-up	Phase I	2016	18		Yes in-vivo	AAV2		Blood	Haemophilia A
Kuopio University Hospital Heart Centre	ReGenHeart: Clinical development and proof of principle testing of new regenerative VEGF-D therapy for cost effective treatment of refractory angina. A Phase II randomised, double-blinded, placebo-controlled study.	Evaluation of the safety and efficacy of catheter mediated AdVEGF-D regenerative gene transfer in patients with refractory angina with no revascularisation option.	2017-000789-31 NCT03039751	Bart's Health NHS Trust/Queen Mary University London	2- In set-up	Phase II	2018	180		Yes in-vivo	Adenovirus		Cardiovascular	Refractory Angina
Adaptimmune	MAGE A10-^{796}T for Advanced NSCLC	A Phase I Dose Escalation Open Label Clinical Trial Evaluating the Safety and Efficacy of MAGE A10-^{796}T in Subjects With Stage IIIb or Stage IV Non-Small Cell Lung Cancer (NSCLC)	NCT02592577	UCLH, Christie	3- Recruiting	Phase I	2015	28	T cells	Yes ex-vivo		Autologous	Cancer	NSCLC
Adaptimmune	AFP-^{332}T in Advanced HCC	A Phase I Open Label Clinical Trial Evaluating the Safety and Anti-Tumor Activity of Autologous T **Cells** Expressing Enhanced TCRs Specific for Alpha Fetoprotein (AFP-^{332}T) in HLA-A2 Positive Subjects With Advanced Hepatocellular Carcinoma (HCC).	NCT03132792	NIHR UCLH Clinical Research	3- Recruiting	Phase I	2017	30	T cells	Yes ex-vivo		Autologous	Cancer	Hepatocellular Carcinoma (HCC)
University College, London	Targeted Stem Cells Expressing TRAIL as a Therapy for Lung Cancer	The aim of the study is to evaluate the safety and anti-tumour activity of MSCTRAIL in addition to chemotherapy in metastatic Non-small cell lung cancer (NSCLC) patients in a Phase I/II clinical trial. In the phase I study, patients will receive cisplatin and pemetrexed on day one followed by MSCTRAIL cells on day 2. This constitutes one cycle of treatment. Each patient will receive 3 cycles of treatment at 21 day intervals. The aim of phase 1 is to estimate the recommended Phase II dose (RP2D) of MSCTRAIL in combination with pemetrexed/cisplatin chemotherapy. During the phase II study patients will be randomised to either the intervention or the control arm of the study. All patients in both arms will receive cisplatin and pemetrexed on day one of treatment. Patients randomised to the intervention arm will receive the recommended dose of MSCTRAIL from Phase I on day 2 whilst those in the control arm will receive a placebo. As this is a single blind trial patients will not know whether they are receiving MSCTRAIL or a placebo product but the clinical team will. The aim of phase 2 is to assess tolerability and preliminary efficacy of MSCTRAIL in combination with pemetrexed/cisplatin chemotherapy.	UCL/14/0453	University College London Hospital	2- In set-up	Phase I/II	2018	46	Mesenchymal stem/stromal cells	Yes ex-vivo	Lentivirus	Allogeneic	Cancer	Adenocarinoma of Lung
Autolus Limited	CD19 /22 CAR T Cells (AUTO3) for the Treatment of B Cell ALL	The purpose of this study is to test the safety and efficacy of AUTO3, a CAR T cell treatment targeting CD19 and CD22 in paediatric or young adult patients with relapsed or refractory B cell acute lymphoblastic leukaemia.		Great Ormond Street Hospital for Children. University College London Hospitals NHS. Royal Manchester Children's Hospital.	3- Recruiting	Phase I/II	2017	50		Yes ex-vivo	Retrovirus	Autologous	Cancer (Haematology)	B cell ALL
Autolus Limited	CD19/22 CAR T Cells (AUTO3) for the Treatment of Diffuse Large B Cell Lymphoma	A Single Arm, Open-label, Multi-centre, Phase I/II Study Evaluating the Safety and Clinical Activity of AUTO3, a CAR T Cell Treatment Targeting CD19 and CD22 Followed by Consolidation With Anti PD1 Antibody in Patients With Relapsed or Refractory Diffuse Large B Cell Lymphoma	2016-004682-11	University College London Hospitals NHS Foundation Trust. The Christie NHS Foundation Trust. Freeman Hospital, The Newcastle upon Tyne Hospitals NHS Foundation Trust	3- Recruiting	Phase I/II	2017	120	T cells	Yes ex-vivo	Retrovirus	Autologous	Cancer (Haematology)	B Cell Lymphoma
Autolus Limited	APRIL CAR T Cells (AUTO2) Targeting BCMA and TACI for the Treatment of Multiple Myeloma	A Single-Arm, Open-Label, Multi-Centre, Phase I/II Study Evaluating the Safety and Clinical Activity of AUTO2, a CAR T Cell Treatment Targeting BCMA and TACI, in Patients With Relapsed or Refractory Multiple Myeloma	2016-003893-42	University College London Hospitals NHS Foundation Trust The Christie NHS Foundation Trust Freeman Hospital, The Newcastle upon Tyne Hospitals NHS Foundation Trust	3- Recruiting	Phase I/II	2017	80	T cells	Yes ex-vivo	Retrovirus	Autologous	Cancer (Haematology)	Multiple Myeloma

Cell and Gene Therapy Catapult clinical trials database 2018

Name of Sponsor	Title	Project Summary	Clinical Database Numbers	United Kingdom Site(s)	Clinical Trial Status	Trial Phase	Year Trial Started	Recruitment Target	Cell Type	Gene Modification/ Gene Therapy	If applicable, type of virus vector used	Autologous/ Allogeneic	Disease Area	Indication
MeiraGTx UK II Ltd	An Open Label, Multi-centre, Phase I/II Dose Escalation Trial of a Recombinant Adeno-associated Virus Vector (AAV2/5-hRKp.RPGR) for Gene Therapy of Adults and Children With X-linked Retinitis Pigmentosa Owing to Defects in Retinitis Pigmentosa GTPase Regulator (RPGR)	This is an open-label phase I/II dose-escalation trial to determine the safety and efficacy of subretinal administration of AAV2 vector in participants with XLRP caused by mutations in RPGR. There is currently no licensed therapeutic treatment for RPGR XLRP. Among a variety of novel experimental strategies that are currently under investigation, gene therapy is considered the most promising. It is hypothesised that, in those subjects with RP associated with mutations in the RPGR gene, localised gene augmentation with a human RPGR-ORF15 variant will result in the production of a biochemically active RPGR-ORF15 protein and thereby facilitate functional and morphological rescue of both rod and cone photoreceptor cells and consequently improved vision.	NCT03252847	Moorfields Eye Hospital NHS Foundation Trust	3- Recruiting	Phase I/II	2017	36		Yes in-vivo	AAV2		Eye	X-Linked Retinitis Pigmentosa
MeiraGTx UK II Ltd	Long-term Follow-up Study of Participants Following an Open Label, Multi-centre, Phase I/II Dose Escalation Trial of a Recombinant Adeno-associated Virus Vector for **Gene Therapy** of Adults and Children With Achromatopsia Owing to Defects in CNGB3	This study is a longer-term follow-up study for patients who participated in a clinical trial of AAV - CNGB3 retinal gene therapy for patients with achromatopsia.The study is designed to collect data on the longer term safety and efficacy up to 60 months following administration.	NCT03278873	Moorfields Eye Hospital NHS Foundation Trust	3- Recruiting	Phase I/II	2017	18		Yes in-vivo	AAV2		Eye	Achromatopsia
University College, London	GO-8: Gene Therapy for Haemophilia A Using a Novel Serotype 8 Capsid Pseudotyped Adeno-associated Viral Vector Encoding Factor VIII-V3	The GO-8 study focuses on assessing safety and efficacy of gene therapy for patients with severe haemophilia A	NCT03001830	Royal Free Hospital London, United Kingdom, NW3 2QG	3- Recruiting	Phase I	2017	18		Yes in-vivo	AAV8		Blood	Haemophilia A
University College, London	A Phase I/II, Open Label, Multicentre, Ascending Single Dose, Safety Study of a Novel Adeno- Associated Viral Vector (FLT180a) in Patients With Haemophilia B	Severe haemophilia B is a bleeding disorder where a protein made by the body to help make blood clot is either partly or completely missing. This protein is called a clotting factor; with severe haemophilia B, levels of clotting factor IX (FIX) (nine) are very low and affected individuals can suffer life threatening bleeding episodes. HB mainly affects boys and men (normally one in every 30,000 males). Current treatment for HB involves intravenous infusions of factor IX as regular treatment (Prophylaxis) or 'on demand'. On demand treatment is highly effective at stopping bleeding but cannot fully reverse long-term damage that follows after a bleed. Regular treatment can prevent bleeding, however can be invasive for patients and also expensive. This research study aims to test the safety and effectiveness of a gene therapy which produces Factor IX protein in the body. The gene will be given using an inactivated virus called "the vector" (FLT180a), in a single infusion. The vector has been developed from a virus known as an adeno- associated virus, that has been changed so that it is unable to cause a viral infection in humans. This "inactivated" virus is further altered to carry the Factor IX gene and to make its way within liver cells where Factor IX protein is normally made. Up to three different doses of FLT180a will be tested, in up to 18 patients with severe haemophilia B. Patients will be recruited from haemophilia centres in the EU and US. Patients will be in the trial for approximately 40 weeks and will undergo procedures including physical examinations, bloods tests, ECGs and liver ultrasounds.	NCT03369444	Royal Free Hospital London, United Kingdom Oxford University Hospital Oxford, United Kingdom	3- Recruiting	Phase I	2017	18		Yes in-vivo	AAV		Blood	Haemophilia B
Audentes Therapeutics	VALENS: A Phase 1/2, Randomized, Open-Label, Ascending-Dose, Delayed-Treatment Concurrent Control Clinical Study to Evaluate the Safety and Preliminary Efficacy of AT342, an AAV8-Delivered Gene Transfer Therapy in Crigler-Najjar Syndrome Subjects Aged 1 Year and Older	This is a Phase 1/2, multinational, open-label, ascending-dose, delayed-treatment concurrent control clinical study to evaluate the safety and preliminary efficacy of AT342 in subjects with Crigler-Najjar aged ≥1 year. Subjects will receive a single dose of AT342 and will be followed for safety and efficacy for 5 years.	NCT03223194	King's College Hospital NHS Foundation Trust London, United Kingdom, SE9 9RS	3- Recruiting	Phase I/II	2017	12		Yes in-vivo	AAV8		Metabolic and Endocrine	Crigler-Najjar syndrome
BioMarin Pharmaceutical	Phase 3 Open-Label Single-Arm Study To Evaluate The Efficacy and Safety of Valoctocogene Roxaparvovec an AAV Vector-Mediated Gene Transfer of Human Factor VIII in Hemophilia A Patients With Residual FVIII Levels ≤1 IU/dL Receiving Prophylactic FVIII Infusions	This clinical trial is being conducted to learn more about a potential treatment (valoctocogene roxaparvovec) for people with severe hemophilia A. This research study will test and confirm the safety and effectiveness of the 6E13 vg/kg dose of the study drug (valoctocogene roxaparvovec) that contains the correct gene to make Factor VIII so that the body can make its own Factor VIII that functions properly. Only one dose of valoctocogene roxaparvovec is being given in this study, and this dose has been previously studied in another clinical trial in patients with hemophilia A. This is a phase 3 study which is meant to show that the study drug is safe and works to help treat hemophilia A. The study will see if liver cells are able to make Factor VIII that functions properly after receiving this study drug. The study will also examine the effects that the study drug has on how much Factor VIII concentrates patients have to inject into their veins and on their bleeding episodes after the study drug has been administered. Finally, the study will see if and how the body responds to the study drug - for example, whether liver cells become inflamed or whether the body makes antibodies (something the immune system makes to protect itself against things like bacteria and viruses) against the vector or the new Factor VIII gene.	NCT03370913	Basingstoke and Northamptonshire; Queen Elizabeth, Birmingham: Addenbrookes; Bart's; Hammersmith; St Thomas'; Churchill; University Hospital Southampton; Royal Cornwall	3- Recruiting	Phase III	2017	40		Yes in-vivo	AAV		Blood	Hemophilia A
oMarin Pharmaceuti	Single-Arm Study To Evaluate The Efficacy and Safety of Valoctocogene Roxaparvovec in Hemophilia A Patients at a Dose of 4E13 vg/kg	This clinical trial is being conducted to learn more about a potential treatment (valoctocogene roxaparvovec) for people with severe hemophilia A. This research study will test and confirm the safety and effectiveness of the 4E13 vg/kg dose of the study drug (valoctocogene roxaparvovec) that contains the correct gene to make Factor VIII so that the body can make its own Factor VIII that functions properly. Only one dose of valoctocogene roxaparvovec is being given in this study, and this dose has been previously studied in another clinical trial in patients with hemophilia A. This is a phase 3 study which is meant to show that the study drug is safe and works to help treat hemophilia A. The study will see if liver cells are able to make Factor VIII that functions properly after receiving this study drug. The study will also examine the effects that the study drug has on how much Factor VIII concentrates patients have to inject into their veins and on their bleeding episodes after the study drug has been administered. Finally, the study will see if and how the body responds to the study drug - for example, whether liver cells become inflamed or whether the body makes antibodies (something the immune system makes to protect itself against things like bacteria and viruses) against the vector or the new Factor VIII gene.	NCT03392974	Queen Elizabeth, Birmingham: Addenbrookes; Bart's; Hammersmith; St Thomas'; University Hospital Southampton; Royal Cornwall	2- In set-up	Phase III	2018	40		Yes in-vivo	AAV		Blood	Hemophilia A
GenSight Biologics, France	Long-term Follow-up of ND4 LHON Subjects Treated With GS010 Ocular Gene Therapy in the RESCUE or REVERSE Phase III Clinical Trials	The goal of this clinical trial is to assess the long-term safety and efficacy of GS010, a gene therapy, and assess the quality of life in subjects with LHON due to the G11778A ND4 mitochondrial mutation and who were treated in the Rescue or Reverse studies.	NCT03406104	Moorfields Eye Hospital London, Greater London, United Kingdom, EC1V 2PD	3- Recruiting	Phase III	2018	74		Yes in-vivo	AAV2		Eye	Leber Hereditary Optic Neuropathy (LHON)
bluebird bio	A Phase 3 Single Arm Study Evaluating the Efficacy and Safety of Gene Therapy in Subjects With Transfusion-dependent β-Thalassemia, Who Have a β0/β0 Genotype, by Transplantation of Autologous CD34+ Stem Cells Transduced Ex Vivo With a Lentiviral βA-T87Q-Globin Vector in Subjects ≤50 Years of Age	This is a single-arm, multi-site, single-dose, Phase 3 study in approximately 15 subjects ≤50 years of age with transfusion-dependent β-thalassemia (TDT), who have a β0 mutation at both alleles of the β-globin (HBB) gene (i.e. β0/β0). The study will evaluate the efficacy and safety of autologous hematopoietic stem cell transplantation (HSCT) using LentiGlobin BB305 Drug Product.	NCT03207009	University College London Hospital London, United Kingdom	3- Recruiting	Phase III	2017	15	CD34 and/or CD133 stem cells	Yes ex-vivo	Lentivirus	Autologous	Blood	Transfusion-dependent β-Thalassemia
GenSight Biologics	Efficacy and Safety of Bilateral Intravitreal Injection of GS010: A Randomized, Double-Masked, Placebo-Controlled Trial in Subjects Affected with G11778A ND4 Leber Hereditary Optic Neuropathy for Up to One Year (REFLECT)	The goal of this clinical trial is to assess the efficacy of intravitreal GS010 compared to placebo intravitreal injection in second affected/not yet affected eyes, at 1-Year post-treatment, utilizing the change from baseline of the best-corrected visual acuity (BCVA) reported with the Log of the Minimal Angle of Resolution (LogMAR), in ND4 LHON subjects with vision loss up to one year	NCT03293524	Moorfields Eye Hospital NHS Foundation Trust, London	2- In set-up	Phase III	2018	90		Yes in-vivo	AAV2		Eye	Leber Hereditary Optic Neuropathy (LHON)
Great Ormond Street Hospital for Children NHS Foundation Trust	Efficacy and safety of a cryopreserced formulation of autologous CD34+ haematopoietic stem cells transduced ex vivo with EFS lentiviral vector encoding for human ADA gene in subjects with severe combined immunodeficiency due to Adenocine Deaminase Deficiency	Lentiviral gene therapy for ADA-SCID. Autologous haematopoietic stem cells transplanted after modification with a lentiviral vector expressing the human ADA gene	2017-001275-23	Great Ormond Street Hospital for Children NHS Trust London,	3- Recruiting	Phase II	2018	10	CD34 and/or CD133 stem cells	Yes ex-vivo	Lentivirus	Autologous	Inflammatory and immune system	Adenosine Derminase Deficiency
AveXis, Inc.	Single-Dose Gene Replacement Therapy Clinical Trial for Patients With Spinal Muscular Atrophy Type 1 (STRIVE-EU)	Phase 3, open-label, single-arm, single-dose, trial of AVXS-101 (gene replacement therapy) in patients with spinal muscular atrophy (SMA) Type 1 who meet enrollment criteria and are genetically defined by a biallelic pathogenic mutation of the survival motor neuron 1 gene (SMN1) with one or two copies of survival motor neuron 2 gene (SMN2). Up to 30 patients < 6 months (< 180 days) of age at the time of gene replacement therapy (Day 1) will be enrolled.	NCT03461289	Great Ormond Street Hospital for Children The John Walton Muscular Dystrophy Research Centre MRC Centre for Neuromuscular Diseases at Newcastle	3- Recruiting	Phase III	2018	30		Yes in-vivo	AAV9		Neurological	Spinal Muscular Atrophy (SMA)
AveXis, Inc.	Pre-Symptomatic Study of Intravenous AVXS-101 in Spinal Muscular Atrophy (SMA) for Patients With Multiple Copies of SMN2 (SPR1NT)	A global study of intravenous AVXS-101 in pre-symptomatic patients with SMA with 2, 3, and 4 copies of SMN2	NCT03505099	Great Ormand Street Children's Hospital (GOSH), London	3- Recruiting	Phase III	2018	44		Yes in-vivo	AAV		Neurological	Spinal Muscular Atrophy (SMA)
Freeline Therapeutics	A Study of Haemophilia B Patients Who Have Undergone Gene Therapy	This clinical study aims to investigate the long-term safety and durability of FIX activity in participants who have been dosed with a new gene therapy product (FLT180a) in earlier clinical studies. Following administration, FLT180a results in production of FIX in the participants liver cells which is then released into the blood stream. The aim is to have the participants own body produce levels of FIX that allow for clotting to occur as normal as would be seen in a non-HB individual. This would remove the need for prophylaxis or on demand treatment following just a single administration of FLT180a.	NCT03641703	Royal Free London NHS Foundation Tust Oxford University Hospitals NHS Foundation Trust	3- Recruiting	Phase II/III	2018	50		Yes in-vivo	AAV		Blood	Hemophilia B

Cell and Gene Therapy Catapult clinical trials database 2018

Name of Sponsor	Title	Project Summary	Clinical Database Numbers	United Kingdom Site(s)	Clinical Trial Status	Trial Phase	Year Trial Started	Recruitment Target	Cell Type	Gene Modification/ Gene Therapy	If applicable, type of virus vector used	Autologous/ Allogeneic	Disease Area	Indication
Autolus Limited	Long-term Follow-up of Patients Treated With Autologous T Cells Genetically Modified	The purpose of this study is to monitor all patients exposed to an existing AUTO CAR T cell therapy, as well as the Sponsor's future autologous T cell products for up to 15 years following their first AUTO CAR T cell therapy infusion.	NCT03628612	Great Ormond Street Hospital for Children NHS Foundation Trust☐ University College London Hospitals NHS Foundation Trust☐ Royal Manchester Children's Hospital The Christie NHS Foundation Trust☐	3- Recruiting	Phase II	2018	500	T cells	Yes ex-vivo	Retrovirus	Autologous	Cancer	Multiple
Bayer AG	Factor VIII Gene Therapy Study in Patients With Hemophilia A	The purpose of this study is to determine the safety and tolerability of the factor VIII gene transfer treatment with BAY 2599023 (DTX201) in individuals with severe hemophilia A.	NCT03588299	St Thomas' Hospital☐ London, United Kingdom, SE1 7EH Manchester Royal Infirmary☐ Manchester, United Kingdom, M13 9WL	2- In set-up	Phase I/II	2018	18		Yes in-vivo	AAV		Blood	Hemophilia A
CRISPR Therapeutics	A Safety and Efficacy Study Evaluating CTX001 in Subjects With Transfusion-Dependent β-Thalassemia	This is a single-arm, open-label, multi-site, single-dose Phase 1/2 study in up to 12 subjects 18 to 35 years of age with transfusion-dependent β-thalassemia (TDT), non-β0/β0. The study will evaluate the safety and efficacy of autologous CRISPR-Cas9 Modified CD34+ Human Hematopoietic Stem and Progenitor Cells (hHSPCs) using CTX001.	NCT03655678	"Research Site", London, United Kingdom	3- Recruiting	Phase I/II	2018	45	CD34 and/or CD133 stem cells	Yes ex-vivo	Electroporation	Autologous	Blood	beta thalassaemia
BioMarin Pharmaceutical Inc	Gene Therapy Study in Severe Haemophilia A Patients With Antibodies Against AAV5 (270-203)	This study is being conducted by Biomarin Pharmaceutical Inc. as an open label, single dose study in order to determine the safety of valoctocogene roxaparvovec (an Adenovirus-Associated Virus (AAV) based gene therapy vector in participants with severe Haemophilia A who also have pre-existing antibodies against AAV5.	NCT03520712	Royal Free Hospital☐ London, United Kingdom University Hospital Southampton NHS Foundation Trust☐ Southampton, United Kingdom	3- Recruiting	Phase I/II	2018	10		Yes in-vivo	AAV		Blood	Bleeding And Clotting Disorders
Ultragenyx Pharmaceutical Inc	Long Term Follow Up to Evaluate DTX301 in Adults With Late-Onset OTC Deficiency (CAPtivate)	Determine the long-term safety of DTX301 following a single intravenous (IV) dose in adults with late-onset ornithine transcarbamylase (OTC) deficiency.	NCT03636438	Queen Elizabeth Hospital, Department of Endocrinology☐ Birmingham, United Kingdom, B15 2TH	3- Recruiting	Phase I/II	2018	12		Yes in-vivo	AAV8		Metabolic and Endocrine	Ornithine-Transcarbamylase Deficiency
GenSight Biologics	Dose-escalation Study to Evaluate the Safety and Tolerability of GS030 in Subjects With Retinitis Pigmentosa (PIONEER)	The objective of this study is to evaluate the safety and tolerability of escalating doses of a gene therapy called GS030-DP (injected study treatment) administered via a single intravitreal injection and repeated light stimulation using a medical device called GS030-MD (stimulating glasses) in subjects with documented diagnosis of non-syndromic Retinitis Pigmentosa	NCT03326336	Moorfields Eye Hospital, London	2- In set-up	Phase I/II	2018	18		Yes in-vivo	AAV2		Eye	Retinitis Pigmentosa (Retinitis)
Mina Alpha Ltd	First-in-Human Safety and Tolerability Study of MTL-CEBPA in Patients With Advanced Liver Cancer (OUTREACH)	This is a First in Human study of a new single agent (MTL-CEBPA) in patients with advanced cancer of the liver. The study is in two parts: dose escalation followed by a dose expansion; both parts of the study will recruit advanced hepatocellular carcinoma patients with cirrhosis. All participants will be refractory to or ineligible for loco-regional therapy including surgery, radiofrequency tumour ablation, transarterial chemoembolisation or sorafenib.	NCT02716012	Birmingham, Cambridge, Beatson, Clatterbridge, Guy's & St Thomas', Imperial, UCL, Newcastle	3- Recruiting	Phase I	2018	51		Yes in-vivo	Non viral - liposomes		Cancer	Adv liver cancer
Sangamo	Ascending Dose Study of Genome Editing by Zinc Finger Nuclease Therapeutic SB-FIX in Subjects With Severe Hemophilia B	The purpose of the study is to evaluate the safety, tolerability and effect on FIX antigen and activity levels of ascending doses of SB-FIX. SB-FIX is an intravenously delivered Zinc Finger Nuclease (ZFN) Therapeutic for genome editing. It inserts a correct copy of the Factor 9 gene into the albumin locus in hepatocytes with the goal of lifelong therapeutic production of the Factor IX clotting factor.	NCT02695160	Glasgow RI, Hammersmith, St Thomas'	3- Recruiting	Phase I	2018	12		Yes in-vivo	AAV2		Blood	Haemophilia B
Oxford BioMedica Plc	Phase I/IIa Study of OXB-102 in Subjects with Parkinson's Disease	The purpose of this study is to evaluate the safety, tolerability and efficacy of OXB-102 in the treatment of subjects with Parkinson's disease	GDC30006795; GDCT0235747	National Institute for Health Research, University of Cambridge Centre for Brain Repar, The National Hospital for Neurology and Neurosurgery, University College London	3- Recruiting	Phase I/II	2018	30		Yes in-vivo	Lentivirus		Neurological	Parkinson's Disease