#### Wednesday 19 June

14:00	Registration desk open Refreshments on Basement Level	Ground Level
15:00- 16:00	BSGCT Annual General Meeting	Lecture Theatre 3
16:00- 18:15	Welcome & Symposium 1: Hot topics Chairs: Peter Andrews, University of Sheffield; Sophia kleine Holthaus, UCL Institute of Ophthalmology	LT3
16:00-16:10	Welcome	
16:10-16:50	INV01 KEYNOTE: Development of cell transplantation for Parkinson's disease Roger Barker, <i>University of Cambridge</i>	_
16:50-17:15	INV02 From bench to bedside: Intrathecal gene therapy for Batten disease Kathrin Meyer, Nationwide Children's Hospital, Ohio	_
17:15-17:40	INV03 Efficient in vivo editing of patient-derived primary human hepatocytes Samantha Ginn, AGCTS/CMRI, Sydney	_
17:40-18:05	INV04 Gene therapy of mtDNA disease Michal Minczuk, <i>University of Cambridge</i>	_
18:05- 18:15	1 minute rapid poster presentations	LT3
19:00- 20:45	BSGCT Welcome Networking Reception Sponsored by Synpromics and Oxford Biomedica Surrey Str	ter Gardens eet, S1 2LH
	Synpromics Oxford Biomedica	

08:15	Registration desk open	Ground Level
08:45- 10:30	Symposium 2: Monogenic/rare diseases Chairs: Laura Ferraiulo, <i>University of Sheffield</i> ; Nora Clarke, <i>Imperial College London</i>	LT3
08:45-09:10	INV05 Gene therapeutics for motor neuron diseases and frontotemporal dementia Mimoun Azzouz, <i>University of Sheffield</i>	
09:10-09:35	INV06 Tay-Sachs and Sandhoff diseases - a rare therapeutic opportunity in the brain Timothy Cox, <i>University of Cambridge</i>	
09:35-10:00	INV07 Gene therapy for pulmonary disorders Uta Griesenbach, Imperial College London	
10:00-10:15	OR01 Onasemnogene abeparvovec gene-replacement therapy for spinal muscular atrophy: From bench to bedside Imran Kausar, AveXis Inc, Bannockburn, IL	
10:15-10:30	OR02 AAV9 gene therapy rescue of an eEF1A2 knockout mouse model Rajvinder Karda, <i>University College London</i>	
10:30- 11:00	Refreshments	Basement Level
11:00- 12:35	Symposium 3 (parallel): Ocular disorders Chairs: Ivana Barbaric, University of Sheffield; Emily Bates, Cardiff University	LT3
11:00-11:25	INV08 A review of the current state of RPE transplantation for treating AMD Pete Coffey, University College London	

11:25-11:50	INV09 Gene therapy for Leber hereditary optic neuropathy Patrick Yu-Wai-Man, <i>University of Cambridge</i>	
11:50-12:05	OR03 The therapeutic potential of AAV-ophNdi1 for the treatment of Leber hereditary optic neuropathy (LHON) Naomi Chadderton, <i>Trinity College, Dublin</i>	
12:05-12:20	OR04 Characterisation of a retinal ganglion cell promoter Sophia Millington-Ward, <i>Trinity College, Dublin</i>	
12:20-12:35	OR05 Effect of human vitreous humour components and neutralising antibodies on transduction activity of recombinant AAV2, AAV5, AAV6 and AAV8 Slawomir Andrzejewski, LVF Ophthalmology Research Centre, Brisbane	
11:00- 12:35	Symposium 4 (parallel): Viral and non-viral vector development Chairs: Uta Griesenbach, Imperial College London; João Cruzeiro, University of Sheffield	LT4
11:00-11:25	INV10 Bacteriophage: From applications in infectious diseases to targeted systemic gene therapy of human cancer Amin Hajitou, Imperial College London	
11:25-11:50	INV11 Therapeutic DNA vaccination: RALA peptide-mediated gene delivery via dissolving microneedles Helen McCarthy, Queen's University Belfast	
11:50-12:05	OR06 Adeno-associated viral vectored delivery of a monoclonal antibody gene against blood-stage malaria Martino Bardell, <i>University of Oxford</i>	

12:05-12:20	OR07 Efficient and scalable production systems for viral vector manufacture Qian Liu, Oxford Genetics Ltd	
12:20-12:35	OR08 Virus-free production of CAR-T cells for the treatment of solid tumors Pavel Simara, International Clinical Research Center, St. Anne's University Hospital Brno	
12:35- 14:00	Lunch & Posters Basement & Gro	ound Level
13:00- 13:30	Stemcell Technologies Symposium Ensuring high-quality hPSC cultures through stabilized maintenance conditions Brian Duff, Stemcell Technologies	LT3
	STEMCELL™	
14:00- 15:50	Symposium 5 (parallel): CNS and autoimmune disorders Chairs: Basil Sharrack, University of Sheffield; Jack Hickmott, Imperial College London	LT3
14:00-14:25	INV12 Autologous haematopoietic stem cell transplantation for severe treatment resistant autoimmune diseases Basil Sharrack and John Snowden, <i>University of Sheffield</i>	
14:25-14:50	INV13 Haematopoietic stem cell gene therapy and inflammation in CNS lysosomal diseases Brian Bigger, <i>University of Manchester</i>	
14:50-15:05	OR09 Long-term neurological correction is sustained in mucopolysaccharidosis IIIB mice following haematopoietic stem cell gene therapy Stuart Ellison, <i>University of Manchester</i>	
15:05-15:20	OR10 Development of novel synthetic promoters for CNS gene therapy Maha Tijani, <i>University College London</i>	

15:20-15:35	OR11 A stem cell model of spinal muscular atrophy (SMA): Assessing combinatorial drug therapies Vinay Kumar Godena, <i>University of Sheffield</i>	
15:35-15:50	OR12 AAV Gene therapy approach for the treatment of Dravet syndrome Juan Antinao Diaz, <i>University College London</i>	
14:00- 15:50	Symposium 6 (parallel): Haematological disorders and immunology Chairs: Kyriacos Mitrophanous, Oxford Biomedica; Shaun Wood, University of Manchester	LT
14:00-14:25	INV14 Harnessing the potential of V61 gamma delta T cells Natalie Mount, GammaDelta Therapeutics, London	
14:25-14:50	INV15 Gene therapy for haemophlia: Progress and problems Edward Tuddenham, <i>University College London</i>	
14:50-15:05	OR13 New strategy of cancer vaccination by a hybrid bacteriophage vector and a malaria vaccine Sajee Waramit, <i>Imperial College London</i>	
15:05-15:20	OR14 Defining the immunological properties of hESC-derived otic neural progenitors in the context of the gerbil auditory neuropathy model Leila Abbas, <i>University of Sheffield</i>	
15:20-15:35	OR15 Use of heterologous vesiculovirus G proteins circumvents the humoral anti-envelope immunity in lentivector based in vivo gene delivery Yasuhiro Takeuchi, University College London	
15:35-15:50	OR16 Gene therapy for thrombotic thrombocytopaenic purpura Robyn Bell. Imperial College London	

15:50- 16:30	Refreshments		Basement Level
16:30- 17:20	Symposium 7: Funding and Regulatory Chairs: Rafael Yáñez-Muñoz, Royal Holloway, University Stuart Ellison, University of Manchester	of London;	LT3
16:30-16:55	INV16 Advanced therapies VC funding funding outlook 201 Dmitry Kuzmin, 4BIO Capital, London	9	
16.55-17:20	INV17 Advance therapy treatment centres Jackie Barry, Cell and Gene Therapy Catapult, London		
17:20- 18:00	Panel session – challenges in clinical transla	tion	
18:00- 19:00	BSGCT Exhibition, Drinks, Posters		Ground Level and Basement
19:30	Conference Networking Dinner Sponsored by Stemcell Technologies After Dinner Speaker: Peter Goodfellow, FRS	Church Stree	utlers' Hall et, S1 1HG
	STEWCELL,"		

## Friday 21 June

08:15	Registration desk open	Ground Level
08:15- 09:00	Refreshments including breakfast rolls	ement Level
09:00- 10:20	Symposium 8: Cancer Chairs: Amin Hajitou, Imperial College London; Jason Halliwell, University of Sheffield	LT3
09:00-09:25	INV18 The promise of iNKT cells as a versatile platform for chimaeric antigen receptor-based immunotherapy of blood cancers Anastasios Karadimitris, <i>Imperial College London</i>	_
09:25-09:50	INV19 Improved replication and selectivity of oncolytic Ad∆∆ and Ad-3∆-A20T promotes targeting to distant lesions in preclinical pancreatic cancer models Gunnel Halldén, Queen Mary University of London	_
09:50-10:05	OR17 Evaluation of the ανβ6 selective precision virotherapy Ad5NULL-A20 as an effective oncolytic virotherapy for pancreatic and triple negative breast cancers James Davies, Cardiff University	
10:05 - 10:20	OR18 Targeting the tumour vasculature with CAR-T cells for treatmer of solid tumours Juan Miguel Sanchez-Nieto, Cell and Gene Therapy Catapult, London	nt
10:20- 11:10	Refreshments Bas	ement Level
11:10- 12:30	Symposium 9: Pulmonary and cardiac disease Chairs: Mustafa Munye, Cell and Gene Therapy Catapult, London; Dylan Stavish, University of Sheffield	LT3
11:10-11:35	INV20 Tissue engineering for cardiovascular repair Richard Jabbour, Imperial College London	_
11:35-12:00	INV21 TACTICAL: A phase I/II trial to assess the safety and efficacy of MSCTRAIL in metastatic lung adenocarcinoma Beth Sage, University College London	_

### Friday 21 June

12:00-12:15	OR19 Immune suppression for xeno-cell transplantation strategies in the porcine animal model Evelyne Demkes, <i>University Medical Center Utrecht</i>	_
12:15-12:30	OR20 Allele specific repair of splicing mutations in cystic fibrosis through AsCas12a genome editing Gianluca Petris, <i>University of Trento</i>	_
12:30- 13:30	Lunch	Basement & Ground Level
13:00- 13:30	Synpromics Ltd Symposium Mastering gene control to enable the next generation of gene medicines Michael Roberts, Synpromics Ltd  Synpromics  The bander in gene control	LT3
13:30- 15:00	BSGCT Special Lecture and Fairbairn Award presentations Chairs: Mimoun Azzouz, University of Sheffield; Evangelia Karyka, University of Sheffield Sponsored by Cancer Research UK Manchester Institute  CANCER RESEARCH MANCHESTER INSTITUTE	LT3
13:30-13:45	Fairbairn 1 OR21 Bacteriophage-mediated systemic gene therapy of diffuse intrinsic pontine glioma through the blood-brain barrier Wenqing Yan, Imperial College London	_
13:45-14:00	Fairbairn 2 OR22 Adipose derived stem cells for cell therapy of Motor Neuron Disease (MND) Yuri Ciervo, University of Sheffield	_

### Friday 21 June

15:05	Concluding remarks and awards ceremony
	Stephan Grupp, Children's Hospital of Philadelphia
	KEYNOTE: Cell therapy for leukaemia: Development and current status of Kymriah
14:30-15:05	INV22
	Thomas Kisby, University of Manchester
	de-differentiated state
	Transient reprogramming of cardiomyocytes to a proliferative
14.10 14.00	OR24
14:15-14:30	Fairbairn 4
	Alexander Baker, Cardiff University
	Adenovirus serotype 26 uses sialic acid bearing glycans as its primary means of infection
	OR23
14:00-14:15	Fairbairn 3