**Wednesday 19 June**

<table>
<thead>
<tr>
<th>Time</th>
<th>Event</th>
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| 14:00   | Registration desk open  
Refreshments on Basement Level                                           |
| 15:00-16:00 | BSGCT Annual General Meeting                                        |
| 16:00-18:15 | Welcome & Symposium 1: Hot topics  
Chairs: Peter Andrews, University of Sheffield;  
Sophia kleine Holthaus, UCL Institute of Ophthalmology |
| 16:00-16:10 | Welcome                                                             |
| 16:10-16:50 | INV01  
KEYNOTE: Development of cell transplantation for Parkinson's disease  
Roger Barker, University of Cambridge |
| 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, University of Cambridge |
| 18:05-18:15 | 1 minute rapid poster presentations                               |
| 19:00-20:45 | BSGCT Welcome Networking Reception  
Sponsored by Synpromics and Oxford Biomedica  
Sheffield Winter Gardens  
Surrey Street, S1 2LH |
Thursday 20 June

08:15  Registration desk open

08:45-10:30 Symposium 2: Monogenic/rare diseases
Chair: Laura Ferraiulo, University of Sheffield; Nora Clarke, Imperial College London

08:45-09:10 INV05
Gene therapeutics for motor neuron diseases and frontotemporal dementia
Mimoun Azzouz, University of Sheffield

09:10-09:35 INV06
Tay-Sachs and Sandhoff diseases - a rare therapeutic opportunity in the brain
Timothy Cox, University of Cambridge

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, University College London

10:30-11:00 Refreshments

11:00-12:35 Symposium 3 (parallel): Ocular disorders
Chair: Ivana Barbaric, University of Sheffield; Emily Bates, Cardiff University

11:00-11:25 INV08
A review of the current state of RPE transplantation for treating AMD
Pete Coffey, University College London
Thursday 20 June

11:25-11:50  INV09
Gene therapy for Leber hereditary optic neuropathy
Patrick Yu-Wai-Man, University of Cambridge

11:50-12:05  OR03
The therapeutic potential of AAV-ophNd1 for the treatment of
Leber hereditary optic neuropathy (LHON)
Naomi Chadderton, Trinity College, Dublin

12:05-12:20  OR04
Characterisation of a retinal ganglion cell promoter
Sophia Millington-Ward, Trinity College, Dublin

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  LT4
Chairs: Uta Griesenbach, Imperial College London;
João Cruzeiro, University of Sheffield

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, University of Oxford
### Thursday 20 June

<table>
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<tr>
<th>Time</th>
<th>Event</th>
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| 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                                                      |
| 13:00-13:30  | Stemcell Technologies Symposium                                       
               Ensuring high-quality hPSC cultures through stabilized maintenance conditions  
               Brian Duff, *Stemcell Technologies*                                    |
| 14:00-15:50  | Symposium 5 (parallel): CNS and autoimmune disorders                 
               Chairs: Basil Sharrack, *University of Sheffield*;  
               Jack Hickmott, *Imperial College London*                             |
| 14:00-14:25  | INV12 Autologous haematopoietic stem cell transplantation for severe treatment resistant autoimmune diseases  
               Basil Sharrack and John Snowden, *University of Sheffield*           |
| 14:25-14:50  | INV13 Haematopoietic stem cell gene therapy and inflammation in CNS lysosomal diseases  
               Brian Bigger, *University of Manchester*                             |
| 14:50-15:05  | OR09 Long-term neurological correction is sustained in mucopolysaccharidosis IIB mice following haematopoietic stem cell gene therapy  
               Stuart Ellison, *University of Manchester*                           |
| 15:05-15:20  | OR10 Development of novel synthetic promoters for CNS gene therapy  
               Maha Tijani, *University College London*                              |
Thursday 20 June

15:20-15:35  **OR11**  
A stem cell model of spinal muscular atrophy (SMA): Assessing combinatorial drug therapies  
Vinay Kumar Godena, University of Sheffield

15:35-15:50  **OR12**  
AAV Gene therapy approach for the treatment of Dravet syndrome  
Juan Antinao Diaz, University College London

14:00-15:50  **Symposium 6 (parallel): Haematological disorders and immunology**  
Chairs: Kyriacos Mitrophanous, Oxford Biomedica; Shaun Wood, University of Manchester

14:00-14:25  **INV14**  
Harnessing the potential of Vδ1 gamma delta T cells  
Natalie Mount, GammaDelta Therapeutics, London

14:25-14:50  **INV15**  
Gene therapy for haemophilia: Progress and problems  
Edward Tuddenham, University College London

14:50-15:05  **OR13**  
New strategy of cancer vaccination by a hybrid bacteriophage vector and a malaria vaccine  
Sajee Waramit, Imperial College London

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, University of Sheffield

15:20-15:35  **OR15**  
Use of heterologous vesiculovirus G proteins circumvents the humoral anti-envelope immunity in lentivirus based in vivo gene delivery  
Yasuhiro Takeuchi, University College London

15:35-15:50  **OR16**  
Gene therapy for thrombotic thrombocytopenic purpura  
Robyn Bell, Imperial College London
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<th>Time</th>
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<tbody>
<tr>
<td>15:50-16:30</td>
<td>Refreshments</td>
<td>Basement Level</td>
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<tr>
<td>16:30-17:20</td>
<td><strong>Symposium 7: Funding and Regulatory</strong></td>
<td>LT3</td>
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<td>Chairs: Rafael Yáñez-Muñoz, <em>Royal Holloway, University of London</em>; Stuart Ellison, <em>University of Manchester</em></td>
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<td>16:30-16:55</td>
<td><strong>INV16</strong></td>
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<td>Advanced therapies VC funding funding outlook 2019</td>
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<td>Dmitry Kuzmin, <em>4BIO Capital, London</em></td>
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<td>16:55-17:20</td>
<td><strong>INV17</strong></td>
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<td>Advance therapy treatment centres</td>
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<td>Jackie Barry, <em>Cell and Gene Therapy Catapult, London</em></td>
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<td>17:20-18:00</td>
<td>Panel session – challenges in clinical translation</td>
<td>Ground Level</td>
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<td>18:00-19:00</td>
<td><strong>BSGCT Exhibition, Drinks, Posters</strong></td>
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<td>19:30</td>
<td><strong>Conference Networking Dinner</strong></td>
<td>Cutlers’ Hall</td>
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<td>Sponsored by Stemcell Technologies</td>
<td>Church Street, S1 1HG</td>
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<td><em>After Dinner Speaker:</em> Peter Goodfellow, FRS</td>
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Friday 21 June

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<th>Time</th>
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<tbody>
<tr>
<td>08:15</td>
<td>Registration desk open</td>
<td>Ground Level</td>
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<tr>
<td>08:15-09:00</td>
<td>Refreshments including breakfast rolls</td>
<td>Basement Level</td>
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<tr>
<td>09:00-10:20</td>
<td>Symposium 8: Cancer</td>
<td>LT3</td>
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<td>09:00-09:25</td>
<td>INV18</td>
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<td></td>
<td>The promise of iNKT cells as a versatile platform for chimaeric</td>
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<td>antigen receptor-based immunotherapy of blood cancers</td>
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<td>Anastasios Karadimitris, Imperial College London</td>
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<td>09:25-09:50</td>
<td>INV19</td>
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<td>Improved replication and selectivity of oncolytic AdΔΔ and</td>
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<td>Ad-3Δ2A20T promotes targeting to distant lesions in preclinical</td>
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<td>pancreatic cancer models</td>
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<td></td>
<td>Gunnel Halldén, Queen Mary University of London</td>
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<td>09:50-10:05</td>
<td>OR17</td>
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<td>Evaluation of the ovβ6 selective precision virotherapy</td>
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<td>Ad5NULL-A20 as an effective oncolytic virotherapy for</td>
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<td>pancreatic and triple negative breast cancers</td>
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<td></td>
<td>James Davies, Cardiff University</td>
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<td>10:05-10:20</td>
<td>OR18</td>
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<td>Targeting the tumour vasculature with CAR-T cells for treatment</td>
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<td>of solid tumours</td>
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<td>Juan Miguel Sanchez-Nieto, Cell and Gene Therapy Catapult, London</td>
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<td>10:20-11:10</td>
<td>Refreshments</td>
<td>Basement Level</td>
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<tr>
<td>11:10-12:30</td>
<td>Symposium 9: Pulmonary and cardiac disease</td>
<td>LT3</td>
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<td>11:10-11:35</td>
<td>INV20</td>
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<td>Tissue engineering for cardiovascular repair</td>
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<td>Richard Jabbour, Imperial College London</td>
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<td>11:35-12:00</td>
<td>INV21</td>
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<td>TACTICAL: A phase I/II trial to assess the safety and efficacy of</td>
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<td>MSTCTR AIM in metastatic lung adenocarcinoma</td>
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<td>Beth Sage, University College London</td>
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Friday 21 June

12:00-12:15  OR19
Immune suppression for xeno-cell transplantation strategies in the porcine animal model
Evelyne Demkes, University Medical Center Utrecht

12:15-12:30  OR20
Allele specific repair of splicing mutations in cystic fibrosis through AsCas12a genome editing
Gianluca Petris, University of Trento

12:30-13:30 Lunch

13:00-13:30 Synpromics Ltd Symposium
Mastering gene control to enable the next generation of gene medicines
Michael Roberts, Synpromics Ltd

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

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

14:00-14:15  Fairbairn 3
OR23
Adenovirus serotype 26 uses sialic acid bearing glycans as its primary means of infection
Alexander Baker, Cardiff University

14:15-14:30  Fairbairn 4
OR24
Transient reprogramming of cardiomyocytes to a proliferative de-differentiated state
Thomas Kisby, University of Manchester

14:30-15:05  INV22
KEYNOTE: Cell therapy for leukaemia: Development and current status of Kymriah
Stephan Grupp, Children’s Hospital of Philadelphia

15:05  Concluding remarks and awards ceremony