

**British Society for Gene and Cell Therapy (BSGCT) Conference  
Royal Holloway, University of London,  
17-19 April, 2013**

**Programme**

**Day 1: Wednesday April 17th 2013**

09:30 - **BSGCT PUBLIC ENGAGEMENT DAY**

15:00 Proudly hosted with



**Windsor Building Auditorium**

*"Gene Therapy and Stem Cells: 21st Century Medicine"*

09:30 - **BSGCT GENE THERAPY FOR INHERITED METABOLIC DISEASE MEETING**

16:00 **First Floor Windsor Building**

15:00 **Conference registration opens - Windsor Building**

16:00 - **BSGCT EDUCATIONAL SYMPOSIUM**

18:00 **Windsor Building Auditorium**

Chairs: **Andrew Baker, University of Glasgow and  
Chris Denning, University of Nottingham**

**Chris Denning, University of Nottingham**

ED 1.1

Using pluripotent stem cells to understand heart disease

**Georgina Ellison, King's College, London,**

ED 1.2

Tissue-specific stem-progenitor cells from the adult mammalian heart and skeletal muscle

**Kyriacos Mitrophanous, Oxford BioMedica**

ED 1.3

Overview of current LV gene therapy clinical trials and challenges ahead


**Len Seymour, University of Oxford**

ED 1.4






Current and future strategies in cancer gene therapy

18:00 **Welcome reception and buffet**

Picture Gallery, Founders Building

19:00	<b>Welcome and opening announcements</b> by Adrian Thrasher (BSGCT President) and Andrew Baker (BSGCT President-Elect) <b>Windsor Building Auditorium</b>	
19:10	<b>SPECIAL GUEST LECTURE: Juan Carlos Izpisua Belmonte, Salk Institute for Biological Studies, California</b> Engineering human somatic and stem cells for cell therapy	SGL 1.1
19:50 - 21:00	<b>FAIRBAIRN AWARD PRESENTATIONS</b> Proudly sponsored by  Chairs: <b>Andrew Baker, University of Glasgow</b> <b>and Adrian Thrasher, Institute of Child Health,</b> <b>University College London</b>	
19:50	<b>Juliette Delhove, Queen Mary University of London</b> Somatotransgenic bioimaging: a novel biosensing platform for in vivo bioimaging	FB 1
20:05	<b>Lynsey Howard, University of Bristol</b> Proangiogenic effect of localised Ad.decoy-mediated miR-15/16 inhibition	FB 2
20:25	<b>Ben Mead, University of Birmingham</b> The neuroprotective and pro-regenerative effects of intravitreal dental pulp stem cell/mesenchymal stem cell transplantation on retinal ganglion cells after optic nerve injury	FB 3
20:40	<b>Ms Ngoc Lu-Nguyen, Royal Holloway-University of London</b> Efficient striatal expression of hGDNF from integration-deficient lentiviral vectors ameliorates 6-OHDA-lesioned rats	FB 4
<b>21:00</b>	<b>“Stumble Inn” and “Medicine” bars open</b> Cheese and biscuits available for delegates from 21:30	
<b>Day 2: Thursday April 18th 2013</b>		
07:15 - 08:15	<b>MEET THE EXPERT OVER BREAKFAST</b> Proudly sponsored by <b>The Hub</b> – pre-registration required	
08:00	<b>Conference desk opens - Windsor Building</b>	
08:30 - 10:15	<b>SYMPOSIUM 1: EMERGING TECHNOLOGIES &amp; VECTOROLOGY</b> Chairs: <b>Stuart Nicklin, University of Glasgow</b> and <b>Paul Kingston, University of Manchester</b>	
08:30	<b>Daniela Moralli, University of Oxford</b> Human artificial chromosomes maintained in human embryonic stem and neuronal cells	S 1.1

09:00	<b>Fulvio Mavilio, Genethon, France</b> Mechanisms and patterns of retroviral insertion	S 1.2
09:30	<b>Andrew Baker, University of Glasgow</b> Manipulation of the vasculature using gene and miRNA approaches	S 1.3
10:00	<b>Proffered paper</b> <b>Richard Harbottle, Imperial College London</b> Permanent genetic modification of dividing cells using episomally maintained S/MAR DNA vectors - the genetic correction of a cancer phenotype in renal tumour cells	S 1.4
<b>10:15 - 10:45</b>	<b>MORNING REFRESHMENTS, POSTERS AND EXHIBITION</b> Windsor Building	
10:45-12:30	<b>SYMPOSIUM 2: STEM CELL ENGINEERING</b> Chairs: <b>Ludovic Vallier, University of Cambridge and Steven Howe, Institute of Child Health, University College London</b>	
10:45	<b>Henrik Semb, Danish Stem Cell Center, Copenhagen University, Denmark</b> Human stem cell technologies	S 2.1
11:15	<b>Bill Skarnes, Wellcome Trust Sanger Centre, Cambridge</b> A library of homozygous mutant stem cells for cellular phenotyping	S 2.2
11:45	<b>Ludovic Vallier, University of Cambridge</b> Liver differentiation and genetic modification of human pluripotent stem cells	S 2.3
12:15	<b>Proffered paper</b> <b>Rafael Yáñez, Royal Holloway-University of London</b> Rescue of mouse Prkdc scid deficiency by transplantation of gene-repaired haematopoietic stem cells	S 2.4
<b>12:30-13:30</b>	<b>LUNCH, POSTERS AND EXHIBITION</b> Windsor Building	
12:30-13:30	<b>BSGCT ANNUAL GENERAL MEETING</b> All BSGCT members welcome <b>Windsor Building Auditorium</b>	
13:30-15:15	<b>SYMPOSIUM 3: METABOLIC &amp; GENETIC DISEASE SESSION 1</b> Chairs: <b>Giulio Cossu, University College London and Laura Bailey, The University of Edinburgh</b>	
13:30	<b>Dimitri Kullman, University College London</b> Gene therapy in experimental focal neocortical epilepsy	S 3.1
14:00	<b>Timo Otonkoski, University of Helsinki, Finland</b> Modelling of monogenic diabetes in hiPSC	S 3.2

14:30	<b>Giulio Cossu, University College London</b> A Phase I/II cell therapy trial for Duchenne muscular dystrophy	S 3.3
15:00	<b>Proffered paper</b> <b>Antonio Trabalza, Imperial College London</b> Dose dependent neuroprotection of VEGF <sub>165</sub> in Huntington's disease striatum	S 3.4
<b>15:15-15:45</b>	<b>AFTERNOON REFRESHMENTS, POSTERS AND EXHIBITION</b> Windsor Building	
15:45-17:30	<b>SYMPOSIUM 4: IN VITRO STEM CELL APPLICATIONS</b> Chairs: <b>Chris Denning, University of Nottingham and Nicole Kane, Institute of Child Health, University College London</b>	
15:45	<b>Christine Mummery, Leiden University Medical Centre, The Netherlands</b> Cardiomyocytes from human pluripotent stem cells in safety pharmacology, drug discovery and disease	S 4.1
16:15	<b>Marc Peschanski, iSTEM, France</b> Harnessing pluripotent stem cell derivatives to decipher mechanisms and identify treatments for monogenic diseases	S 4.2
16:45	<b>SPECIAL GUEST:</b> <b>Konrad Hochedlinger, Harvard Stem Cell Institute</b> Understanding cellular reprogramming and pluripotency	S 4.3
17:15	<b>Proffered paper</b> <b>Gábor Földes Imperial College London</b> Key differences in hypertrophic signalling in hESC- and hiPSC-derived cardiomyocytes	S 4.4
<b>17:30-19:00</b>	<b>DRINKS, POSTER JUDGING AND SPONSOR PRESENTATIONS</b> Windsor Building - <i>with music from Vintage Cocktail</i> 1: BIA Separations 2: Cellular Dynamics International 3. iThera Medical 4: Rett Syndrome Research Trust UK	   
19:00	<b>CONFERENCE DINNER</b> Proudly sponsored by <b>After dinner speaker:</b> <b>Professor Patrick Maxwell, University of Cambridge</b> Pre-registration required <b>Founders Dining Hall, Founders Building Fairbairn and Poster Presentation results will be announced.</b>	 <b>Sangamo BioSciences</b>
<b>"Stumble Inn" and "Medicine" bars open after the conference dinner</b>		

## Day 3: Friday April 19th 2013

07:30 - **BSGCT BOARD MEETING**

08:30 This meeting is open to BSGCT Executive members.  
Breakfast will be provided.

**08:00 Conference desk opens - Windsor Building**

08:30 - **SYMPOSIUM 5: CELLULAR GENE THERAPY FOR CANCER**

10:15 Chairs: **Hans Stauss, University College London and Roua Alsubki, Institute of Child Health, University College London**

08:30 **Ton Schumacher, The Netherlands Cancer Institute, Amsterdam** S 5.1  
Towards personalized gene and T cell therapies in melanoma

09:00 **Hans Stauss, University College London** S 5.2  
Improving T cell therapy of cancer

09:30 **Chiara Bonini, Hospital San Raffaele, Milan** S 5.3  
TCR gene editing to treat haematological malignancies

10:00 *Proffered paper* S 5.4  
**Hong Zhan, Institute of Child Health, University College London**  
First-in-man use of T cells engineered to express a HSVTK-CD34 sort-suicide gene

**10:15- MORNING REFRESHMENTS, POSTERS AND EXHIBITION**

**10:45** Windsor Building

10:45- **SYMPOSIUM 6: METABOLIC & GENETIC DISEASE SESSION 2**

12:30 Chairs: **Keith Foster, University of Reading and Marlene Carmo, Institute of Child Health, University College London**

10:45 **Mimoun Azzouz, University of Sheffield** S 6.1  
SMN replacement gene therapy for spinal muscular atrophy: clinical development

11:15 **Keith Foster, University of Reading** S 6.2  
Engineering adeno-associated virus vectors to deliver full length dystrophin

11:45 **Steve Hart, Institute of Child Health, University College London** S 6.3  
Progress and challenges in gene therapy with nanocomplexes

12:15 **Stephen Hyde, University of Oxford** S 6.4  
Gene therapy for cystic fibrosis: from basic research to clinical impact

<b>12:40-13:30</b>	<b>REGULATORY SESSION</b> <b>Pre-registration required. Lunch will be provided</b> Windsor Building Ground Floor Seminar Room 0-05	
13:30 - 15:15	<b>SYMPOSIUM 7: STEM CELL THERAPIES</b> Chairs: <b>Georgina Ellison, King's College, London and Waseem Qasim, University College London</b>	
13:30	<b>Robin Ali, University College London</b> Stem cell therapy for retinal degeneration	S 7.1
14:00	<b>Phil Newsome, University of Birmingham,</b> Stem cell therapies for liver disease	S 7.2
14:30	<b>Georgina Ellison, King's College, London</b> Stem cell therapies: heart	S 7.3
15:00	<i>Proffered paper</i> <b>Dafni Moschidou, Imperial College London</b> Potential of human fetal chorionic stem cell therapy for Alport Syndrome	S 7.4
<b>15:15-15:45</b>	<b>AFTERNOON REFRESHMENTS, POSTERS AND EXHIBITION</b> Windsor Building	
15:45-17:30	<b>SYMPOSIUM 8: GENE THERAPY FOR CANCER</b> Chairs: <b>Mary Collins, University College London and Cian M.McCrudden, Queen's University, Belfast</b>	
15:45	<b>Alan Melcher, University of Leeds</b> Oncolytic viruses for cancer - from laboratory to clinic and back again	S 8.1
16:15	<b>Iain McNeish, Institute of Cancer Sciences University of Glasgow</b> Oncolytic adenoviruses for ovarian cancer	S 8.2
16:45	<b>Mary Collins, University College London</b> Regulating dendritic cell signalling to improve vaccination	S 8.3
17:15	<i>Proffered paper</i> <b>Steven Mo, University of Oxford</b> Coating of adenovirus type 5 with cleavable PEGylated gold nanoparticles for enhanced protection and circulation	S 8.4
<b>17:30-17:40</b>	<b>CLOSE OF CONFERENCE - BSGCT PRESIDENT</b> Fairbairn and Poster Presentation certificates and prizes will be presented.	