“We aim to provide an opportunity to discuss and debate gene and cell therapy research with scientists, patients, journalists and clinicians, and to think about the impact that this research has on society and you”

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<td>Welcome, Simon Waddington, University College London</td>
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<td>What is the British Society for Gene Therapy? Len Seymour</td>
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<td>What is gene therapy? Louise Collins, Kings College London</td>
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<td>What is stem cell therapy? Nadia Kallinikou, University College London</td>
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<td>How can gene therapy improve the lives of those affected with brain disease? Eric Kremer, Institut de Génétique Moléculaire de Montpellier</td>
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<td>How gene therapy cured our son, Alexander Carol and Colin Locke</td>
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<td>Fanconi...hope: from ‘Saviour Sibling’ to gene therapy Thomas Carroll</td>
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<td>Gene therapy in the media and public opinion Sarah Norcross</td>
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Louise Collins  
*King’s College London*

Louise began her research in Gene Therapy with a PhD from the Institute of Child Health, UCL in 1998 and has since remained in this fascinating field. Her research interests are in vector technology, focussing on the development of non-viral DNA vectors, in particular using synthetic peptides. In addition to the laboratory research, she lectures to undergraduates on the subject of Gene Therapy. Currently Louise juggles her life between work and looking after her two small children – not always easy, but fun! ‘Gene Therapy’ has been given a bad name due to media scaremongering and fantastical and often incorrect film scripts. The scientists are always the bad guys. As research scientists in this field it is vital that we represent the other side of the story. Over the years Louise has always sought the chance to present the subject to both school children and non-specialist audiences. She is very pleased to be given the opportunity to be part of the BSGT Public Engagement Day this year.

Nadia Kallinikou  
*PhD student, University College London*

She has a BSc in Biomedical Sciences and a Masters degree in Human Molecular Genetics. She has been involved in research projects both in Oxford University and the University of Patras, Greece. She is particularly interested in the biology and function and expansion of haematopoietic stem cells, which she has been researching since 2006, in Dr Kwee Yong’s group, at UCL. Her research is in the field of stem cell transplantation, which is a treatment for patients suffering from diseases such as lymphoma and leukaemia. Successful treatment is dependent on the number of stem cells given to the patient. Nadia’s research aims to discover how it maybe possible to achieve successful stem cell transplantation with smaller numbers of stem cells.
**Eric Kremer**  
*Director of Research Inserm, Montpellier, France*

The core theme of his laboratory is the study of the adenovirus family. Human adenoviruses normally cause subclinical symptoms, but in sporadic cases they can be lethal in infants and immunocompromised patients. He is using the inherent properties of canine adenovirus, to generate gene transfer vectors that may be used to treat neurodegenerative disorders. He also wants to understand how adenoviruses interact with cell receptors and how they travel through the cell into the nucleus. His laboratory collaborates with others throughout Europe, Asia, Australia and North America and he recently obtained a 4.5 million euro grant to make gene transfer tools to understand and treat neurodegenerative diseases. More information can be found at [www.braincav.eu](http://www.braincav.eu)

**Sarah Norcross**

Sarah is director of the Progress Educational Trust (PET), and Commissioning Editor of its free weekly email news digest, commentary service and website BioNews. She is also a member of the National Genetics Education and Development Centre’s Steering Group. She chaired and coordinated the steering group of clinicians, scientists, patient support groups and medical research charities which fed into policy for the Human Fertilisation and Embryology Act 2008 as it passed through both Houses of Parliament. She continued in this role during the implementation of the Act by the UK Government’s Department of Health and the Human Fertilisation and Embryology Authority. As well as ensuring that the administrative side of PET and BioNews run smoothly, together with Sandy Starr Sarah also organises PET’s public engagement events. She regularly attends conferences either as a speaker or with a view to commissioning comment pieces for BioNews. Previously she worked as a Barrister carrying out advocacy in the civil and criminal courts in Manchester. For more information see [www.bionews.org.uk](http://www.bionews.org.uk)
Steven Howe  
**Senior research associate, Wolfson Centre for Gene Therapy, UCL Institute of Child Health**

Steven gained his BSc degree in Biology at the University of Bristol, specialising in molecular genetics and cellular biology. Subsequently, he earned his PhD at Imperial College, investigating the potential of artificial chromosomes for gene therapy of genetic disorders such as cystic fibrosis. Steven has worked at the UCL Institute of Child Health since 2001, contributing to successful clinical trials led by Prof. Adrian Thrasher, assessing gene therapy for different disorders of the immune system. His research interests fall into two broad areas; the development of viral vectors for gene delivery and somatic gene therapy, as well as the manipulation of blood stem-cell biology to improve treatment of cancers and the outcome of bone marrow transplantation.

Thomas Carroll  
**Chairman of Fanconi Hope Charitable Trust**

Thomas Carroll is father to a five year old son affected by Fanconi Anaemia (FA), is a founder member of both the Fanconi Hope Charitable Trust and the UK Fanconi Anaemia Clinical Network, aswell as being Co-Editor of the publication UK Standards of Care in FA (Feb, 2009). His family’s personal FA story has been presented in parliament during the debate leading up to the passing of the Human Fertilisation and Embryology Act 2008 and in addition has been part of a recent BBC documentary on ‘Saviour Sibling’. He is also a consultant neurosurgeon at Sheffield specialising in treatment of skull base tumours. Fanconi Hope Charitable Trust, with the support of its Patron, the Duchess of Devonshire, raises awareness of FA, a genetic stem cell disorder that causes bone marrow failure and predisposes to cancer. The charity aims to promote FA as a candidate disorder for future gene therapy. More information can be found at [www.fanconi.org.uk](http://www.fanconi.org.uk)
Len Seymour
President of the BSGT, 2004-2009

‘Gene therapy’ means many different medical strategies. What unifies the approach, however, is that all of the treatments are based on ideas arising from our genes. This is a rapidly growing field of research and it is very important that scientists make the effort to communicate their dreams with the rest of society so that everyone can understand what the future of genetic medicine can bring. Today’s event is an important step in BSGT’s intrepid journey in the jungle of public awareness and we are asking you to tell us where there are gaps in understanding about gene therapy and where the Society should focus its energies. BSGT is always keen to increase its lay membership and we encourage you to join us in promoting inspiring and well-regulated science.

Gene therapy is a new scientific field that tries to take advantage of recent progress in many different scientific disciplines – notably in genetics, but also in cell biology, virology, biochemistry and medicine. This means that for effective progress with gene therapy we need input from people with expertise in a variety of different sciences. We so need to have close interaction with doctors, who will transfer new ideas into clinical studies, and with patients who will ultimately benefit from our research. To bring all of these skills together, in 2003 a group of UK scientists decided to form the ‘British Society for Gene Therapy’, specifically to improve the quality of gene therapy science in the UK by promoting discussion and collaboration, and also to promote public awareness of what we, as scientists, are trying to achieve.
Carol and Colin Locke  
*Parents of Alexander Locke*

In 2004 Alexander Locke was diagnosed with X-linked SCID. Rushed to Great Ormond Street Hospital in London he was eventually treated by the application of gene therapy. This was presented as an alternative to receiving a conventional bone marrow transplant that was essentially unavailable. His parents, Carol and Colin, took time away from their work to look after Alexander whilst he was in hospital and when he was subsequently discharged. A life changing experience, the family is very grateful for the choice of treatment, and subsequent outcome, gene therapy provided. Without gene therapy it is almost certain Alexander would have died. Although still receiving antibody replacement therapy, Alexander is a happy, outgoing 6-year old who is full of life, much to his parents' relief. For their part, Carol and Colin support the Jeans for Genes charity and, as part of this effort, have appeared in publications and on TV a number of times, most recently as part of the BBC series Visions of the Future.

Charles Coutelle  
*Emeritus Professor, Imperial College London*

Charles Coutelle, MD DSc. is an Emeritus Professor of Gene Therapy at the National Heart and Lung Institute, Imperial College London. He has qualified in Medicine and Biochemistry and worked in several fields of Biochemistry and Human Molecular Genetics including gene expression, gene mapping, DNA diagnosis, and in the last 15 years, gene therapy for human genetic diseases. In particular he has investigated the feasibility and implications of gene therapy in utero as an approach to prevent early onset manifestation of severe genetic conditions.
Simon Waddington  
*Lecturer, University College London*

In 1922 the first diabetic received insulin protein from ox pancreas. Thirty six years later, in 1958, Frederick Sanger determined the primary structure of insulin. In the same years Francis Crick first proposed the central paradigm that DNA is the blueprint for proteins, such as insulin. This meant that we would be able to use genes to cure diseases…

…it’s now fifty-one years and counting and we still don’t have gene therapy for Type I diabetes or, for that matter, malaria, schizophrenia, high blood pressure…

…but over the past ten years we have been seeing encouraging results with gene therapy for immune deficiencies, inherited blindness and some cancers.

Are we starting to crack it, at last?

Renee Watson  
*Wats.on Consultancy*

Renee Watson trained as a biochemist in Australia before setting off to explore the world. She discovered the UK seven years ago and since then has worked at the interface between science and the public. Renee has been involved in the establishment of the National Translational Cancer Research Network, an International Malaria Research Network and the National Patient Safety Agency. Renee helped to establish the British Society for Gene Therapy and was a Board member between 2004 - 2006. After having her first child in 2006 Renee decided she wanted more flexibility so set up a consultancy company that helps organisations “do public engagement” and provides training for researchers. Renee is trained in classical ballet, loves working on her allotment and remains as excited by science today as she has always been.
Sponsorship

This meeting is hosted by the Primary Immunodeficiency Association and Jeans for Genes. The meeting has also received support from Fanconi Hope.

The Primary Immunodeficiency Association exists to support people living with primary immunodeficiencies and disorders of the immune system. They liaise with clinicians and immunologists, fund relevant research and campaign for the rights of their members in the UK. For more information see www.pia.org.uk.

Jeans for Genes provides funding for the care and support of children with genetic disorders and their families. They also help to fund research into the genes responsible and the development of effective treatments and cures. For more information see www.jeansforgenes.com.

Fanconi Hope is a registered national charitable trust set up by parents of Fanconi Anaemia (FA) affected children and clinicians with an interest in supporting a national database, promoting public awareness and encouraging research in Fanconi anaemia. For more information see www.fanconi.org.uk.