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Special Focus Issue on the Annual Meeting of the British Society for Gene and Cell Therapy

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THIS SPECIAL ISSUE OF *Human Gene Therapy* is published to coincide with the Annual Meeting of the British Society for Gene and Cell Therapy (BSGCT) taking place in June 2015 at the University of Strathclyde Technology Innovation Centre in Glasgow, United Kingdom. The UK gene and cell therapy community has been at the forefront of international research in the field since its inception, highlighted by the award of 3 of the 12 *Human Gene Therapy* Pioneer Awards for work in gene and cell therapy to UK-based researchers. Robin Ali of University College London Institute of Ophthalmology was recognized for his high-impact translational research into rare inherited blindness.[1,2] Adrian Thrasher, past president of BSGCT, of University College London Institute of Child Health is a leader in the field of immunodeficiency disorders, conducting pioneering trials in young patients and deciphering much of the underlying basic science in the development of gene therapy in this arena.[3–6] Amit Nathwani leads development of clinical gene therapy for hemophilia and has been at the forefront of recognizing the potential application of novel adeno-associated virus serotypes in this arena through translational research.[7–10] Of course these recent pioneers in the field of gene and cell therapy are the latest contributors to the long and proud scientific tradition of the United Kingdom. In fact, from Charles Darwin's work in the 1800s, which recognized that species evolved through adaptation via natural selection, to the work of Watson, Crick, and Franklin at the University of Cambridge, which led to the resolution of the double-helix structure of DNA in the 1950s, and latterly the creation of Dolly the Sheep in the 1990s, UK research into genetics and genetic engineering has always been at the forefront of scientific research.

Although there is natural synergy between the two research areas, the development of the gene therapy research field generally predates that of stem cell therapy. Gene therapy of course has had its high and low points. At the present time and after much hype in the early days, gene therapy is in the ascendancy again, with clear success in the clinic for a range of serious diseases, significant investment from the pharmaceutical industry, and the relatively recent EU marketing license awarded for the first gene therapy medicine, Glybera.[11–13] With early recognition of the unique challenges in converting experimental gene therapies into the clinic, the United Kingdom was at the forefront of shaping regulatory policies in this area. The UK government's Department of Health formed the Gene Therapy Advisory Committee (GTAC), a committee of scientific and medical experts and independent lay members who reviewed submissions for conducting gene therapy clinical trials in the United Kingdom. As well as providing ethical oversight and authorization for clinical trials, they also provided an important and respected scientific advisory function for researchers designing clinical trials to take gene therapies from the lab to the clinic. GTAC has now been dissolved and its functions embedded into the National Research Ethics Service (NRES) of the NHS, highlighting gene therapy's continuing maturation and acceptance into the mainstream clinical setting in the United Kingdom.

Moreover, with the rapid evolution of the cell therapy field, both gene and cell therapies are now classified as advanced medical therapies and are regulated by the UK Medicines and Healthcare Products Regulatory Agency (MHRA), working under EU directives from the Committee for Advanced Therapies at the European Medicines Agency (CAT). Importantly, the advisory aspect first developed for gene therapy through GTAC is still in place for translation of stem cell therapies to the clinic, with the formation of a one-stop shop for advice on regenerative medicines. Therefore, the lessons learned through the history of the development of gene therapy are well placed to support a careful and cautious translation of stem cell therapy from the lab to the clinic. Of course there are still challenges ahead for gene therapy, in particular, bottlenecks in routine availability of GLP and GMP gene therapy vector manufacturing facilities, highlighting that work is still to be done in order to further support and consolidate the field. Therefore, the cell therapy field is well placed to continue learning from the gene therapy field as we move forward.

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The 2015 Annual Scientific Meeting of BSGCT has a program of internationally renowned speakers delivering a broad range of topical science focused on gene and cell therapy, from basic science through to clinical trials. Furthermore, state-of-the-art sessions focused on genome editing and RNA therapeutics highlight the synergy between the gene and cell therapy field. BSGCT has also worked hard to involve junior scientists in the Annual Scientific Meeting, recognizing their importance for the future growth of the research community. This has seen initiatives at the level of invited presentations to junior scientists, including prestigious abstract prizes targeted at PhD and first postdoctoral position researchers, as well as involvement in conference organization, chairing sessions, and abstract reviewing. These transferable skills offer junior scientists the chance to become fully engaged with BSGCT at the outset of their careers. The annual conference is preceded by a Public Education Day, which is provided for local school pupils to come and meet scientists and to learn about gene and cell therapy, further inspiring and stimulating the next generation of researchers. This year the annual scientific meeting also incorporates two one-day satellite meetings. One satellite meeting is focused on inherited metabolic diseases and has become a regular feature of the BSGCT calendar over the past few years. Also, and for the first time, this year BSGCT has partnered with the British Society for Cardiovascular Research to deliver a meeting on Regenerative Medicine for Cardiovascular Disease. This latter meeting has been designed to dovetail with the annual conference to maximize opportunities for networking among delegates. Moreover, the meeting will bring together the three British Heart Foundation–funded UK Centres of Regenerative Medicine. Overall, this comprehensive schedule is designed to facilitate timely updates on rapid advances in gene and cell therapy while acting as a forum for discussion, collaboration, and networking in order to further strengthen the links between the two research fields.

This special issue of *Human Gene Therapy* publishes a varied collection of primary research and review articles to coincide with the annual meeting of BSGCT. All the articles are by active BSGCT members or invited speakers and highlight the excellence and breadth of research in the United Kingdom and those contributing to our meeting. We hope you enjoy the issue and we hope you are planning a sunny week in Glasgow to enjoy the science on offer at BSGCT 2015 and to network and collaborate with peers and colleagues.

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