**The British Society for Gene and Cell Therapy**

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**UK perspective**

The British Society for Gene and Cell Therapy (BSGCT) was founded in 2003 with the goal of advancing the science and practice of gene therapy for the public benefit. As we approach our twentieth anniversary, we remain firmly committed to an aim which is quickly becoming a clinical reality. Gene and cell therapies are formally known as Advanced Therapy Medicinal Products (ATMPs) in the European Union. Technically, the definition of ATMP includes products based on genes, cells or tissues. However, our field of expertise also covers short, chemically synthesized nucleic acids (oligonucleotides) that can alter gene function, which are quickly becoming an important part of the medical armamentarium. Together with the most novel technologies like mRNA delivery, exosomes, genome and base editing, these therapeutics offer avenues of treatment for previously intractable genetic diseases as well as approaches to treat common diseases.

The first gene therapy trials were conducted in the early 90s and since then the number of clinical studies has grown into the thousands. Marketing approvals took some time to arrive, but Gendicine (an adenoviral vector against cancer) received the first one, in China, in 2003. Glybera (an adeno-associated viral vector to treat lipoprotein lipase deficiency) received the first European approval in 2012, but commercially was only used to treat one affected person. After this slow start, oligonucleotides, viral vectors, autologous (from the person affected) and allogeneic (donor-derived) cultured cells, and genetically modified T-cells and haematopoietic stem cells have been steadily licensed. Their relative importance is increasing fast: in the round of recommendations for marketing approval published on 16th October 2020 by the European Medicines Agency (EMA, the EU regulator), four out of the ten drugs with positive recommendations were gene or gene cell therapies. Of particular note, our field has developed most vaccines available against COVID-19, whether based on modified mRNA or recombinant viral vectors, allowing mankind to fight successfully the latest worldwide pandemic.

Not surprisingly, advanced therapeutics are a priority area in the UK and present significant opportunities for health benefit and economic growth. The worldwide gene and cell therapy market has been estimated to reach $21bn/year by 2025, with $716m of gene therapy venture capital funding invested since 2010 (Advanced Therapies Manufacturing Taskforce Report).

We have therefore entered a very exciting phase, where gene and cell therapy drugs are becoming mainstream treatments as well as crucial industrial products. However, the production and delivery of advanced therapeutics is complex and therefore expensive, and significant challenges lie ahead. To allow timely adoption of a significant drug pipeline, every stage in the therapeutic development and delivery process will require innovation, including pre-clinical and clinical studies, manufacturing, approval, pharmaceutical logistics, clinical delivery and reimbursement (the negotiation of a price between manufacturer and NHS or insurer that allows the manufacturer access to the market). In the UK, adoption through healthcare providers such as the NHS requires prior approval by the National Institute for Health and Care Excellence (NICE), a very complex and lengthy process based on cost/benefit analyses which can ultimately limit access, not unusually leading to disappointment for people affected and their families. Fair, realistic and transparent pricing by manufacturers, and novel evaluation methods by healthcare agencies, will be required to ensure that gene and cell therapies become universally available, but also that profits from these innovative technologies support future R&D. In addition, due to the explosive growth of our industry, skill shortages are threatening to become a major bottleneck in further advancing gene and cell therapies, which needs to be urgently addressed through Higher Education, Apprenticeship and Training Programmes.

BSGCT has had major impact in many of these areas. For example, the society lobbied successfully to secure a dedicated UK funding call for Innovation Hubs for Gene Therapy, allowing the creation of a network of three dedicated facilities to advance clinical development of new genetic treatments (<https://www.lifearc.org/news/2021/new-network-of-gene-therapy-hubs>). BSGCT has also been very active promoting the creation of training opportunities in gene and cell therapy, to help address the skills shortage. ATAC (Advanced Therapies Apprenticeship Community, <https://advancedtherapiesapprenticeships.co.uk>) and ATSTN (Advanced Therapies Skills Training Network, <https://www.atskillstrainingnetwork.org.uk>) are two excellent examples of these efforts in the UK.

**BSGCT activity**

BSGCT has around 400 members, grouped in the following categories: Professional academics (including postdoctoral scientists and academic/clinical fellows), Industry scientists, Postgraduate students, Undergraduate and Masters students, and Non-professional/Retired members. To promote inclusion, membership fees are maintained very low, and waived for the last two categories.

BSGCT’s major scientific activity is the annual research conference, to which attendance is promoted through highly subsidised registration fees. We aim to alternate one-day and three-day conferences, but the cycle has been recently disrupted by the ongoing COVID pandemic. Following a highly successful three-day meeting in Sheffield in June 2019, in 2020 we were meant to host the joint ESGCT/BSGCT conference in Edinburgh, but this was postponed and will now be held in October 2022. In 2021 ESGCT is holding a joint virtual congress with all the European national societies, including BSGCT, between 19-22 October. We are looking forward to this resumption of the conferences in 2021.

To better support specific focus areas, BSGCT currently has four sub-committees: Early-Career Development and Collaboration (ECDC), Public Engagement, Communication and Promotion, and Clinical Translation and Regulatory (<https://www.bsgct.org/About/Committees.aspx>). BSGCT considers a priority the career development of younger members of the society, the so-called Early Career Researchers (ECRs). The society reserves three board places for ECRs, who are selected by the board among candidate members and serve a two-year term, with the possibility of being re-selected for a second term. To engage ECRs at all levels, BSGCT ensures that they participate fully in board activities, and in the organization and delivery of the annual research conference, including membership in the Local Organizing Committee, revision of abstracts, chairing of sessions and prize awards. The ECDC sub-committee organises a yearly, free Early Career Development and Collaboration event, the latest being held at the Institute for Child Health, London on 29th November 2019. At these events ECR delegates from around the UK participate in various activities centered around career opportunities, including academia, industry and spin out companies. They learn grant writing skills from funders and can attend CV workshops, as well as having networking opportunities. To overcome COVID-related restrictions, the ECDC subcommittee has organised online training and careers-based talks. Most recently BSGCT has launched a mentoring scheme for ECRs, to provide them with an independent connection in the field. The scheme is designed to pair mentees with a mentor that will support them by drawing on their professional and personal experience, listening to them, and providing information and encouragement. Finally, the society has dedicated prizes for ECRs at the annual conference, particularly the long-standing Fairbairn Award (<https://www.bsgct.org/Scientists--Students/Fairbairn-Award.aspx>).

BSGCT’s Public Engagement sub-committee flagship is the highly successful yearly Public Education Day, normally attracting around 300 registrations from local schools and the general public. At this event we aim to raise awareness of gene and cell therapy, provide an information forum and inspire the next generation of scientists. In addition to this event, BSGCT participates in relevant outreach events orchestrated by other organisations, such as Royal Holloway University of London’s annual Rare Disease Day event (<https://royalholloway.ac.uk/rdd>). In 2021, BSGCT’s sponsorship of this event subsidised the production and shipping to participating schools of one box of resources per child, allowing students to perform hands-on activities at home despite the virtual format imposed by COVID-19-related restrictions. To promote social mobility and diversity in Science, Technology, Engineering and Maths (STEM), in partnership with In2Science (<https://in2scienceuk.org/>) BSGCT yearly supports four gifted pre-university students from low-income families undertaking 2-week summer projects in research laboratories, to gain valuable work experience prior to going to university. In addition, the society awards two eight-week BSGCT undergraduate Summer Research Studentship bursaries for extended work experience of university students.

The Communication and Promotion sub-committee uses online and social media tools to engage members and non-members alike, with a strong emphasis in outreach. BSGCT maintains an up-to-date website including educational resources at <https://www.bsgct.org/>, sends a termly newsletter to members, hosts a regular blog (<https://www.bsgct.org/Education/BSGCT-Blogs.aspx>), and has highly successful Twitter (@\_BSGCT), Facebook (BSGCT) and LinkedIn (BSGCT) accounts. The most recent blogs are on COVID-19 Vaccine-Induced Immune Thrombotic Thrombocytopenia, and Long-Lasting Analgesia Via Targeted *in vivo* Epigenetic Repression. BSGCT also hosts an annual Science Writing Competition for gene and cell therapy outreach communication pieces written by students (postgraduate and undergraduate) and research scientists (pre- and post-doctoral). Publication on the BSGCT website and cash prizes are guaranteed for the winners and other selected pieces.

Finally, BSGCT’s Clinical Translation and Regulatory Affairs sub-committee aims to facilitate the uptake of gene and cell therapies in the clinic by providing advice, information and a discussion forum for stakeholders. Most recently BSGCT has set up a “Stakeholder Panel” including representatives from government agencies, large pharma, SMEs, startups, regulators, funding agencies and patient representatives, to provide advice and guidance to the society in this critical area of activity, and to connect relevant UK stakeholders.

To conclude, gene and cell therapies are becoming a clinical reality but to ensure the realisation of their full potential significant challenges must be overcome. With this goal in mind, BSGCT is always open to feedback and participation from members and other stakeholders, and encourages joint initiatives with other like-minded societies.

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