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The European Society of Gene and Cell Therapy: A Nearly 30-Year Endeavor to Make Gene Therapy a Clinical Reality

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A BRIEF HISTORY

When traveling back in history to the early days of gene therapy, September 14, 1990, marks a key event. French Anderson, Michael Blaese, and colleagues started the first gene therapy clinical trial for a genetic disease. They decided for adenosine deaminase deficiency-severe combined immunodeficiency (ADA-SCID) as a disease to treat and—due to technical limitations—on T lymphocytes as target cells for their effort to make the first tiny steps toward proving the concept of gene therapy.

Inspired by this effort and by the spirit in the scientific community, only 7 months later (April 1991) the first international symposium on gene therapy in Europe was organized in France, hosted by the gene therapy pioneers Michel Boiron and Odile Cohen-Haguenauer. It attracted ~250 participants. Shortly thereafter, in January the next year, the first gene therapy society was founded, the European Working Group on Human Gene Transfer and Therapy, which was renamed into European Society of Gene and Cell Therapy (ESGCT) in 2007. This change was made to better reflect the tight connection between these two fields and their impact on the development of innovative treatment options in oncology and for hitherto untreatable monogenetic disorders.

In these past 29 years, the field has experienced ups and downs before finally reaching the clinic with the first market-approved gene therapies. Europe was on the forefront being the first to approve a gene therapy in the Western world. So far, 10 gene and cell therapies have received market approval in Europe, and more are expected soon. Although the first gene therapy in the Western world that received market approval was a local *in vivo* gene therapy for lipoprotein lipase deficiency, the gene therapy for ADA-SCID, the starting point of the journey, was the first *ex vivo* gene therapy for a monogenetic disease to be approved, which happened 26 years after initiation of the first clinical trial.

OUR MISSION

Through all these years, our society has followed its aim of promoting basic and clinical research in gene therapy, cell therapy, and gene-based vaccine development. ESGCT is carrying out its mission in stimulating the exchange of scientific knowledge and expertise and in fostering understanding of gene and cell therapies with all its aspects and related fields of research.

ESGCT facilitates an informed dialogue between the scientific community, policy makers, and society as a whole and serves as a professional adviser to stakeholders in gene and cell therapy as well as for regulatory bodies in Europe.

ESGCT is committed to the encouragement and support of young investigators entering the field through training and other activities specifically aimed at students and individuals early in their careers in cell and gene therapy.

ESGCT promotes collaborations between and within clinical settings, academic institutions, and the pharmaceutical and biotechnology industries. From building partnerships between industry through sponsorships and exhibitions at its congresses and organizing commercialization and clinical trial workshops, the society is at the center of efforts to make gene and cell therapy a clinical reality.

ESGCT is truly international, with a primary focus on Europe. The close interaction with the European national gene therapy societies and working groups is reflected among others by joint activities such as the ESGCT annual meetings, which rotates each year between different European countries and is organized in conjunction with the respective national societies (Fig. 1). In parallel, our advanced lecture course, termed ESGCT Spring School, is traveling through Europe and is co-organized with the national societies of the hosting country. This close interaction is particularly visible in the coronavirus disease 2019 (COVID-19) pandemic and with the upcoming 2021 ESGCT congress, which is a unique event since eight national societies have jointly co-organized the congress together with us. A further

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Figure 1. Plenary session of the 2019 ESGCT Meeting in Barcelona. Color images are available online.

highlight is the current special issue of *Human Gene Therapy*, the official journal of ESGCT and all national gene therapy societies in Europe. In addition to these close bounds within Europe, ESGCT closely interacts with the American Society of Cell and Gene Therapy (ASGCT) and the Japanese Society for Gene and Cell Therapy.

DEVELOPMENTS IN THE FIELD

As already mentioned, over the past three decades, the field has experienced impressive ''ups'' but also severe ''downs.'' Unperturbed by these sometimes difficult situations, clinicians and scientists from various areas who believed in the concept of gene therapy moved the field forward toward the current point at which gene therapy is entering the clinic. The full potential of gene therapy in making changes in the lives of patients and their families has not yet been fully realized, but compelling evidence of the long-term potency of this innovative treatment option is clearly emerging.

The major challenges that are lying ahead are the need to improve efficacy and safety of current cell and gene therapies and to facilitate the establishment of gene and cell therapy as an integral part of modern medicine. With regard to latter, ESGCT is contributing by developing

Figure 2. Molecular Mingle party at the 2019 ESGCT Meeting in Barcelona. Color images are available online.

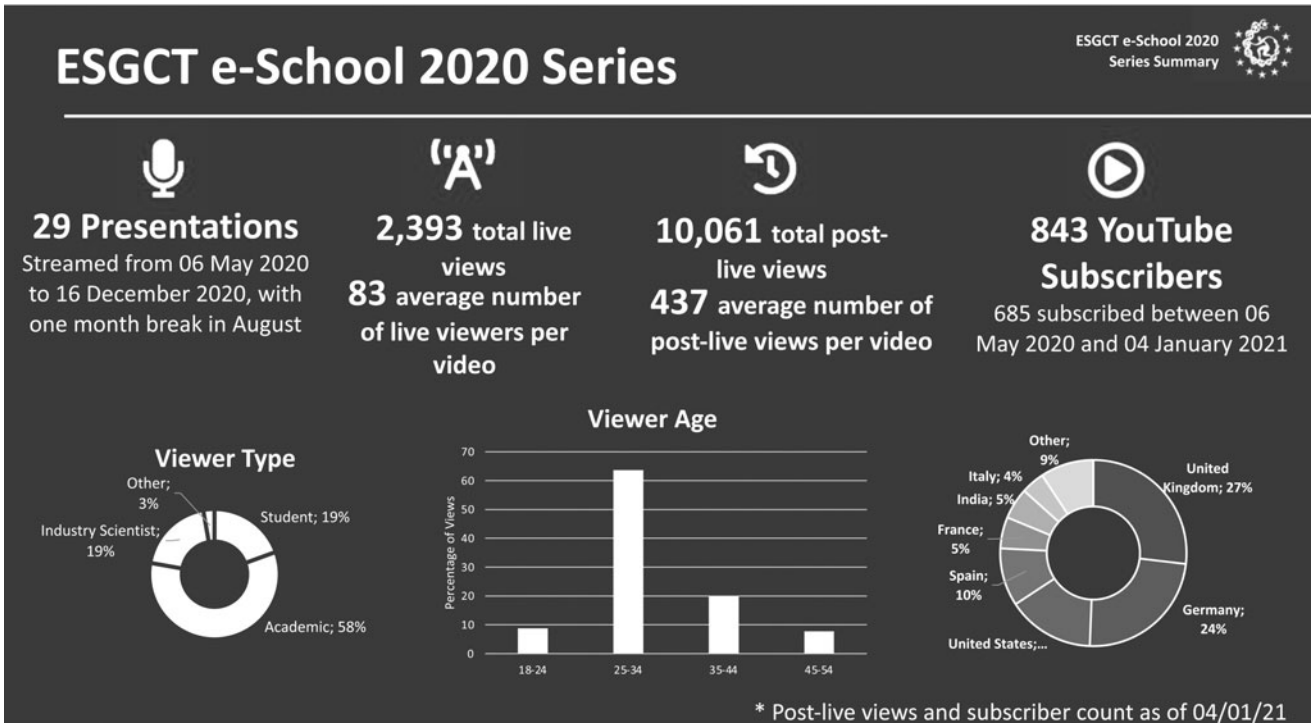

Figure 3. Color images are available online.

information hubs for the lay public, by interacting with patient organizations and regulatory bodies, and by the launch of a working group consisting of all stakeholders in the field. The working group is dedicated to identifying hurdles, both scientific and political, for the transfer of (academic) gene therapies into clinical reality and to propose possible solutions to overcome these hurdles.

Gene and cell therapy-based treatments are currently very costly and require an infrastructure that is not available in all countries, and thus it is also our responsibility to raise awareness and to support efforts to make them available for low- and middle-income people and impoverished countries.

ESGCT ACTIVITIES

ESGCT organizes a wide range of activities. Its annual congress brings together scientists working in the fields of gene and cell therapy from throughout Europe and around the world. The congress is typically held in October in a major European city and provides a platform for highlighting the latest research and techniques through the scientific sessions, which include keynote lectures, invited speakers, poster sessions, and exhibitions. The congress provides an environment conducive to developing relationships and collaborations. It also typically includes a public day, where key concepts in gene and cell therapy along with the latest research are presented in the local language to a general audience, as an integral part of the meeting.

As already mentioned, when the congress is held in a European country with a national gene therapy society, the congress is planned in collaboration with that national society. For example, the Spanish Society for Gene and Cell Therapy was our partner when organizing the 2019 ESGCT congress in Barcelona, a congress that attracted ~2,000 delegates active in the field (Figs. 1 and 2). Alternatively, we collaborate with closely related scientific societies, such as the International Society for Stem Cell Research in 2016.

This year, our annual congress (October 19–22) will be a virtual event for the first time, due to the ongoing COVID-19 pandemic. The virtual congress will provide the same high caliber of speakers and topics for which ESGCT has become known, plus opportunities for networking and establishing new connections between delegates, institutions, and industries. This special year is calling for unique activities and we are delighted that the ESGCT 2021 annual meeting is jointly organized with eight national gene and cell therapy societies from across Europe. Having held no congress at all in 2020, ESGCT is particularly looking forward to reconnecting with members, experts, sponsors, and exhibitors alike, then hopefully returning to in-person meetings in 2022.

To support the continuous supply of new talent entering the field, since 2016 ESGCT has organized an annual Spring School, an advanced lecture course aimed at students and young investigators who are embarking on their journeys in gene and cell therapy. The Spring School is an

intensive training course and provides the opportunity to interact with current and future leaders in the field. As training and education of our next-generation scientists are close to our heart, ESGCT is offering this course for free. Its unique spirit makes our ESGCT Spring School a popular event that is next scheduled to take place in Athens in April 2022.

The society also recognizes inspiring, long-term dedication, and progress in the field through its prestigious Outstanding Achievement Award (OAA) and Young Investigator Award (YIA). Many OAA and YIA recipients of the past decades have contributed substantially to define the state-of-the-art therapies and continue to make creative contributions, moving the field forward at the nexus of basic and translational research, regulatory advances, and clinical development toward ultimate marketing authorization approval and making a tangible difference in patients' lives.

The ESGCT board runs a program for two student members to join the society's board for a fixed term to help organize activities targeted at students and early career researchers. In the past year, a success for the current student board members has been their ''Ask the Expert'' webinar program, giving a unique opportunity to gain sound career advice from leading scientific experts. The program is due to continue in autumn 2021.

Over the past 18 months, ESGCT inaugurated its e-School and e-Seminar events that again are dedicated to educating a wide community on all aspects of cell and gene therapy (e-School; Figure 3) and on emerging topics (e-Seminars) such as the COVID-19 pandemic and gene editing through lectures given by current leaders in the field.

The society has initiated other activities to further engage its members and the wider community, including patients and members of the public. These initiatives include a heat map that shows the location and details of gene and cell therapy research in Europe, blogs written by the society's members, and news items accessible to nonspecialists available on the ESGCT website.

Along the same line, we have teamed up with the ASGCT to provide the most updated information and resources for patients and the public, which can be accessed through the ESGCT website.

As the society approaches its 29th anniversary, ESGCT is as proactive and vibrant as ever, adapting to new circumstances and supporting further exciting developments in gene and cell therapy. It is determined to follow in the tradition of those original pioneers and is inspired by the successes already achieved for patients in a range of diseases—and the enormous potential to treat many more.