Molecular Therapy
Editorial

ASGCT 2023—Gene therapy is becoming medicine

The 26th annual meeting of the American Society of Gene Therapy (ASGCT) took place in Los Angeles in May 2023. This year's meeting celebrated the precision that gene therapy has achieved, ranging from gene editing to specific targeting of cancer cells by receptor-engineered lymphocytes. Gene editing in particular took center stage this year during the Presidential Symposium. Jennifer Doudna explained the principle of gene editing using the CRISPR system and its applications in treatment of human diseases, while David Liu enlightened us on the ever-increasing precision of these evolving technologies and expansion of the toolkit to base and prime editors that do not require making DNA breaks. The strong interest in this area was also well reflected by the abstracts presented at the meeting. Gene editing and correction were among the top-performing abstract categories, accounting for 14% of all abstract submissions and 16% of all oral abstract presentations. Moreover, gene editing and correction are expanding into the clinic, as demonstrated by Haydar Frangoul, who focused on the highly promising outcomes of a clinical trial on gene editing for the treatment of transfusion-dependent β-thalassemia and severe sickle cell disease. Furthermore, J. Keith Joung received the society's Outstanding Achievement Award for his contributions to the development of nucleases for gene editing applications.

Despite the strong interest in gene editing, gene delivery technology still dominated large segments of the meeting, with approximately 170 oral presentations on the adeno-associated viral (AAV) platform. In fact, AAV, which has arguably dominated the annual meeting for decades, accounted for 25% of all abstract submissions. AAV was also featured during the Presidential Symposium, with Hichem Tasfaout and Defne Amado discussing the potential of AAV to treat such diverse diseases as Duchenne muscular dystrophy and amyotrophic lateral sclerosis, respectively. Making AAV safer for clinical translation was also a popular topic during oral abstract sessions, with talks focused on the use of capsid engineering to reduce immune responses to AAV, characterization of anti-AAV9 monoclonal antibodies derived from patients treated with Zolgensma, and the use of capsid engineering, machine learning, and other techniques to improve vector tropism.

Reflecting the maturity of the field, gene and cell therapy clinical trials and regulatory considerations were popular topics. The nuances of the accelerated approval pathway for gene and cell therapies were covered by a lively panel discussion that touched on the FDA's considerations for accelerated approvals, the details of the Skysona (elicel) approval, and the use of surrogate endpoints in clinical trials. The Gene and Cell Therapy Trials in Progress featured eight clinical trials for a variety of diseases, including Duchenne muscular dystrophy, HIV, and Canavan disease, among others. Other sessions covered critical issues such as payment models for gene and cell therapies and patient access, which are becoming a source of concern as more gene and cell therapies enter the market.

Overall, the 2023 annual meeting broke multiple records, with nearly 8,000 attendees (the 2022 meeting was the first to exceed 7,000 attendees), 270 exhibitors, and 1,698 abstracts. The commercial sector had a very strong showing, with 17 Industry-sponsored Symposia focused on topics such as targeted delivery of gene and cell therapies and challenges in manufacturing, 18 Exhibitor Showcases, 5 Tools and Technology Forums (featuring 31 presentations by various companies), and 1 Startup Spotlight (featuring presentations from 9 startups). Industry-sponsored content at the annual meeting has continued to grow in recent years, demonstrating that gene and cell therapy products have firmly established their place in modern medicine. Recent approvals of gene therapies for hemophilia, bladder cancer, and dystrophic epidermolysis bullosa, among others, pay testimony to this development. Therefore, technology development, manufacturing, and clinical development of gene and cell therapy tools and drugs represent the perhaps most prevalent themes at the ASGCT meeting for years to come.

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