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CELL THERAPY STRATEGIES

The path to successful commercialization of cell and gene therapies: empowering patient advocates

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Abstract

Often, novel gene and cell therapies provide hope for many people living with incurable diseases. To facilitate and accelerate a successful regulatory approval and commercialization path for effective, safe and affordable cell and gene therapies, the involvement of patient advocacy groups (PAGs) should be considered early in the development process. This report provides a thorough overview of the various roles PAGs play in the clinical translation of cell and gene therapies and how they can bring about positive changes in the regulatory process, infrastructure improvements and market stability.

Key Words: *affordability, cell and gene therapies, clinical trials commercialization, patient advocacy, regulation, reimbursement*

In recent years, cell and gene therapies, particularly immunotherapies for cancer and gene therapy applications for severe combined immunodeficiencies have crossed over from mere research procedures to highly valuable and effective therapies [1]. Most interestingly, personalized therapies, in which prevention, diagnosis and treatment modalities are tailored to target specific types of diseases based on individual patient variability using factors such as individual patient characteristics (health status, age, medical history), genetic profile and their environment, have received a lot of attention [2]. In light of all these developments, engaging patients and their families proactively in their care can positively affect treatment outcome. However, illness and the stress associated with caring for a loved one can impair judgment, and patients are in need of support so that they can effectively assess the benefits and risks of available novel therapies [3]. Patient advocacy groups (PAGs) are therefore particularly valuable and can play several critical roles in this process. PAGs are groups of stakeholders seeking to inform patients of their rights and providing them with requisite information for informed decisions that affect their health and health care choices; additionally, they can play an important role in translating scientific and medical information into a lay-friendly format. For cell and gene therapies, these groups have been lobbying research organizations and agencies to provide new therapeutic modalities, especially for rare diseases, and help patients and their families become involved in decision-making processes.

Researchers face pressure from the public to develop cell therapies that will reach the clinic within a short time period [4,5]. However, the field of cell and gene therapy research has been subject to significant commercialization challenges, for reasons related to high development costs, heterogeneity of reimbursement and volatile market conditions, among others [6]. Although for many cell and gene therapy–based innovations, the question of whether these technologies can be commercialized at reasonable costs is a major concern, there are also several other aspects that are often not discussed but can influence the successful commercialization of these products. Patient advocates can contribute greatly to addressing these issues and be catalysts in accelerating the commercialization of these products and their adoption into the market, particularly if few patients are affected, and profit-driven, mainstream product commercialization would

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not be easily achievable. The International Society for Cellular Therapy Commercialization Committee recently met with PAGs from around the world (Joseph P. Roberson Foundation, USA; Genetic Alliance UK, United Kingdom; Lung Foundation Australia) to discuss the several ways patient advocacy can influence the success of bringing cell and gene therapies to patients (Table I).

Capturing the power of big data

The amount of data available today in the biotechnology field is unprecedented. This has been triggered by significant technological advances, such as whole genome sequencing, the generation and sharing of large amounts of clinical trial data and the rise in patient numbers and registries [7]. Taking advantage of the rapidly advancing field of bioinformatics generating insights from these data, developers are increasingly able to transform the way cell and gene therapies, and other innovations, are discovered, developed and marketed [8]. Moreover, pricing strategies and shaping new business models [9] are types of efforts that have the potential to benefit from the analysis of large sets of data. It is therefore important that the trust of patients, who may be inherently reluctant to share their data and participate in clinical translational research, will be gained. Transparency and patient ownership of the data they contribute will be central to establishing this trust relationship. PAGs are best positioned to initiate discussions about data sharing and taking an active part in bringing both parties—patients and developers—closer to reaching common understanding and trust that will enable developers to capture the advantages of big data aimed toward improving the success rate of novel therapeutics. For instance, a program titled Big Data for Patients (BD4P) has been introduced by the Reagan-Udall Foundation to provide specialized training for patients and advocates in the imminent discipline of data science (www.reaganudall.org/our-work/bd4p). The program aims to equip PAGs with the knowledge and tools they need to effectively communicate with different stakeholders regarding the use of big data in health care. Such strategies will also enable drug manufacturers to incorporate patient-centered strategies into all stages of clinical research where targeted therapies are lacking.

Combating the threat of unauthorized cell-based interventions

PAGs can play a vital role in stopping unauthorized or even dangerous cell-based interventions [10], often found in "stem cell tourism," by stressing the need for strengthening appropriate and proportionate regulatory oversight and enforcement, and perhaps by helping develop a system of international governance that oversees clinical cell and gene therapy research and treatment. At the same time, it would be important to facilitate patient access to tested and authorized novel therapies in their own countries [11] and allow for the timely, equitable and sustainable reimbursement of safe and efficacious cellular therapies. Although several PAGs have raised concerns about the introduction of the REGROW Act in the United States, which could possibly weaken Food and Drug Administration (FDA) regulations and oversight of experimental stem cell therapies (www.michaeljfox.org/foundation/news-detail.php?mjff-signs-letter-opposing-the-regrow-act), patient advocates could play an important role in obtaining a balance between accessibility of therapies and regulatory oversight. PAGs could participate in discussions with the FDA, the European Medicines Agency (EMA) and other regulatory agencies worldwide to reach this balance. PAGs could also lead initiatives for increasing awareness of the potential risks associated with stem cell tourism and provide education to patients and the public about the harms of such practice and how to spot and report unauthorized stem cell clinics [12]. Most important, it is essential today, more than ever, for PAGs to help manage the therapeutic hopes and, at the same time, the psychological distress of patients suffering from the many diseases that cell and gene therapies are potentially able to treat. PAGs are trusted mediators and have largely remained immune from the criticism placed on industrial drug developers. The sharing of personal narratives from patients and families and ways they have found to handle and manage the mental, emotional and medical aspects of specific, often devastating diseases forms a new set of knowledge, different from what health professionals might be able to offer. Thus, PAGs become a trusted voice that could more effectively pass on the message that research demonstrating treatment safety and efficacy is needed, which could be facilitated and achieved by a well-thought-out regulatory system [13]. PAGs from a range of diseases, where cellular therapies may be effective treatment modalities, should therefore come together, deliberate and issue a joint statement explaining the need to reach a balance between accelerating the delivery of novel therapies to patients and having rigorous research and regulatory oversight in place. Greater coordination is needed to create a more powerful voice, if such a message is to be heard.

Influencing the translational process of novel therapeutic approaches

Taking advantage of the extraordinary reach of online medical information, patients today have more knowledge and influence in the drug development process than ever before. Because many cellular therapy approaches address rare diseases, patient engagement is