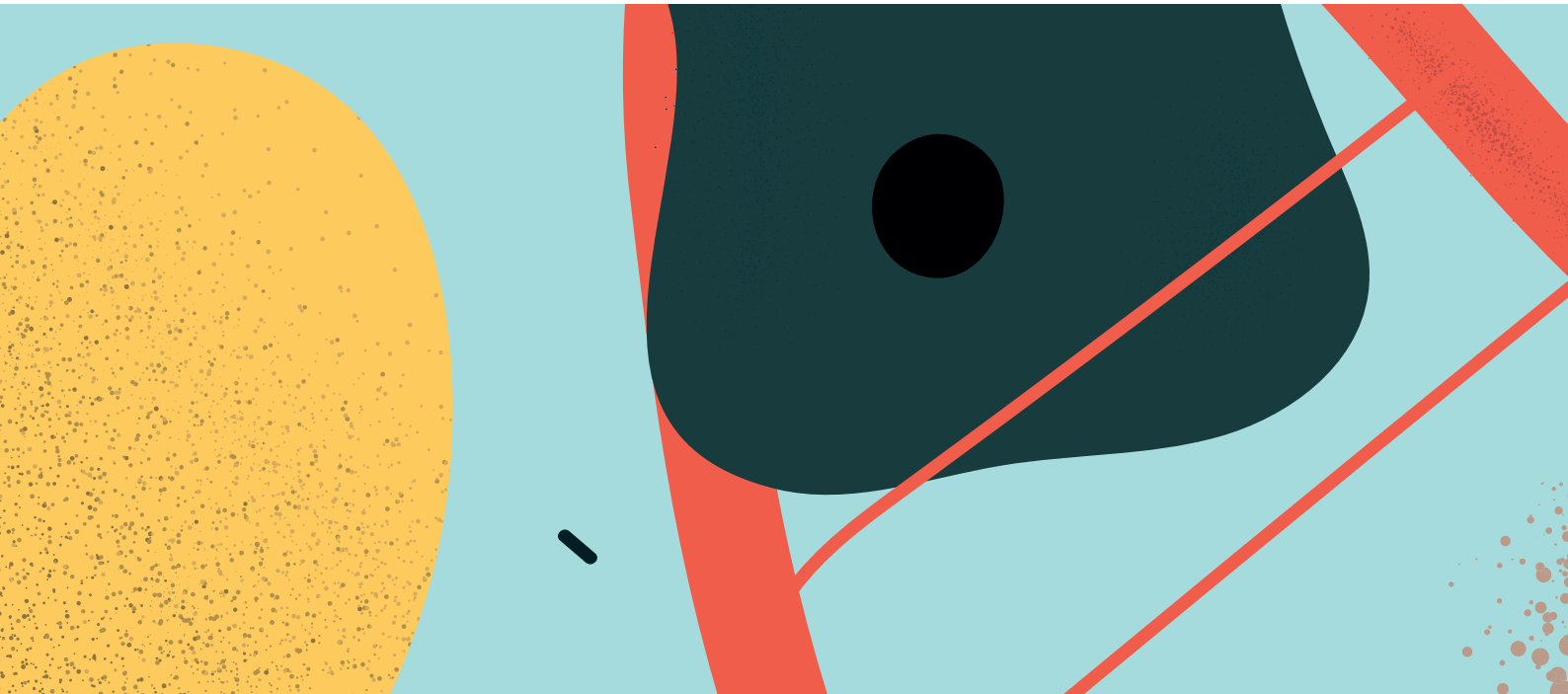
International Alliance of Patients' Organizations

IFPMA

IAPO-IFPMA toolkit on

CELL AND GENE THERAPIES



EXECUTIVE SUMMARY

Cell and gene therapies are innovative technologies in the field of healthcare that have the potential to bring great benefits to patients and society overall. New advances in these exciting therapies offer the potential to transform medicine and our ability to treat many intractable diseases. IFPMA and IAPO have joined efforts to shed light over the science behind cell and gene therapies as well as highlight their potential and some of the challenges that need to be addressed to ensure access
and uptake of these revolutionary treatments.

Although they both work in different ways, cell and gene therapies are fast developing fields of biomedical research that aim to treat diseases by modifying our genetic and cellular makeup. Cell therapy is the administration of living cells for the treatment of a disease. One of such treatments is CAR-T therapy, which aims to re-program the patient's own T-cells so that they are able to detect and destroy cancer cells in the patient's body. Gene therapy works by correcting for errors in the way that the patient's genes are working. This can be done either by adding a correctly functioning copy of the gene to the cells, by blocking the faulty gene, or by correcting the error in the patient's copy of the gene. This is achieved by introducing genetic material into a patient's cells with the help of a carrier so that the effect of the treatment may be permanent after a single administration. These treatments have the ability to halt the progress of a disease or alleviate the underlying cause of a disease.

Cell and gene therapies are still relatively uncharted fields. On this new frontier of medicine, the pharmaceutical industry is pioneering new and efficient methods not only on drug discovery and development, but also on how therapies are tested, regulated and provided to the patients who need them. The compelling science and severity of the conditions studied mean that clinical trials, product manufacturing, post-approval regulatory requirements and means of administration require new ways of working together to bring these therapies to patients.

As cell and gene therapies are gaining momentum, this raises questions around where they will fit in the current pharmaceutical landscape. Conventional medicines which make up the majority of treatments available today, mainly consist of chemically-derived small molecules and biologic medicines. In the case of biologic medicines, the therapeutic molecule is made from living organisms and has a greater complexity, but their mechanism of action is based on the same pharmacodynamic principles as most small molecules. Most conventional therapies are typically used to manage diseases, mitigate symptoms and relieve pain, whereas the goal of cell and gene therapy is to target the exact cause of the disease to stop it from recurring, ideally after a single treatment. Although a single, possibly curative treatment for a disease is favorable, administration of cell and gene therapy are more complex compared to conventional medicines which are mostly available at the pharmacy. A gene or a cell cannot be delivered through a pill: instead, the patient will need to be treated at qualified healthcare centers with trained professionals. This highlights the importance of strengthening healthcare systems to ensure access to and safety of these therapies.

It cannot be denied that there is great promise in these therapies, yet, it is important to point out that they should not be viewed as a wonder cure-all. Like any medical treatment, they present their own unique challenges and benefits.

Cell and gene therapy can provide help and hope to a huge number of patients, patients for whom alternative treatments options are very limited and whose quality of life and life expectancy are often very low. However, these patients generally suffer from one of many specific diseases, which requires developing a specific approach. The complexity of these diseases creates challenges for developing and testing potential treatments. For example, some genetic diseases are caused by mutations in a single gene, while others, such as many cancers, are a result of mutations in
multiple genes. Considering that currently available gene therapy options are limited to treating diseases caused by a single gene mutation, a very small number of patients can benefit from gene therapy treatments at the moment. Further complexities such as expressing the gene in the right tissue, at the right level, for the right amount of time highlight the expertise required for successfully manufacturing and delivering these treatments.

Industry is working closely together with regulatory authorities to make sure clinical trials for cell and gene therapies are fit for purpose and designed in a way that can reliably assess them for safety and efficacy. This means innovative trial designs often need to be created and/or utilized. Because of these targeted clinical trials, cell and gene therapies are often approved as safe and effective based on a streamlined evidence base, safeguarded by specific follow-up measures in the post-approval phase. These include detailed risk management plans, including education and
accreditation of treatment centers to ensure that not only the treatment, but also the administration and follow-up, meet the highest safety standards. This means that cell, specifically gene-modified cell such as CAR-Ts, and gene therapies can only be provided at accredited treatment centers.

Furthermore, manufacturing of these therapies is very complex. Training highly specialized personnel, ensuring the integrity of the supply chain and conducting detailed quality and safety testing of each individual product, all contribute to ensuring that the therapy is available to as many patients as possible. Industry is working on not only meeting regulatory requirements, but going beyond to ensure the highest possible standard of treatment for patients.

Realizing the full potential of cell and gene therapies for helping the vast number of patients suffering from diseases that lack viable treatment options requires creating a supportive regulatory, intellectual property, reimbursement and pricing environment, which is tailored to the unique opportunities and specific challenges of these treatments.

Stakeholders are invited to use this brochure as an introduction to cell and gene therapies and as a patient-friendly directory for additional information. We aim to provide support to patients, policy-makers and healthcare professionals who want to know more about these therapies and have a better understanding of their role in ensuring that cell and gene therapies can deliver on their full potential.

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