# Cell and Gene Therapies are set to Revolutionise Healthcare: Here's How

The first chemotherapy drugs were discovered by mistake – during World War II – when humans were accidentally exposed to mustard gas. At that time, the only other treatments for cancer were radiotherapy and surgery. But these weren't necessarily promising for the treatment of all forms of cancer, especially cancers that had progressed.

Scientists were recruited to study the symptoms of people that were exposed to copious amounts of mustard gas. Based on their research, mustard gas agents were transformed into chemotherapy drugs used for the treatment of cancers. Chemotherapy is still the standard first line of cancer therapy for many people with cancer. Because chemotherapy doesn't only target cancer cells, they are associated with many side effects including diarrhoea and hair loss.

But with a focus on patient centricity, the life science industry as we know it is changing.

Over the last couple of years, we have seen personalized medicine take the centre stage. One particular class of products, called Advanced Therapeutic Medicinal Products (or ATMPs, for short), is gaining a lot of interest from scientists, doctors, researchers, investors, and patients, alike. Put simply, these therapies utilize cells, tissues, and/or gene approaches to develop products that can repair, generate, or replace faulty cells that cause diseases.

ATMPs, including cell and gene therapies (CGTs), have emerged as a groundbreaking solution, offering long-term symptom relief and even complete cures for patients who were previously considered incurable. These therapies have the potential to shift the paradigm in chronic and rare diseases, transitioning from mere disease management to achieving full recoveries. What was once a distant aspiration for scientists is now becoming a tangible reality, as various CGTs receive approvals based on promising results from clinical trials.

But with a focus on patientcentricity, the life science industry as we know it is changing.

**A Case for CGTs: Diffuse Large B-Cell Lymphoma**

**What is Diffuse Large B-Cell Lymphoma?**
Diffuse Large B-Cell Lymphoma (DLBCL) is a type of cancer affecting the lymphatic system. It is characterized by its aggressive nature and rapid growth in the lymph nodes, liver, bone marrow, spleen, and other organs. DLBCL arises from the production of abnormal B lymphocytes, which normally play a crucial role in combating infections. However, in DLBCL, these abnormal cells fail to fully mature and accumulate in the lymph nodes and other organs, compromising the patient's ability to fight infections effectively.

Regardless of the chosen medical approach, DLBCL treatment is often intense and requires frequent hospital visits over a short period. Coping with the diagnosis and treatment of this cancer can be challenging for patients, leaving them feeling fatigued and drained, particularly during and after treatment.

**Standard Treatment for Diffuse Large B-Cell Lymphoma**
Standard treatment for Diffuse Large B-Cell Lymphoma (DLBCL) is determined by various factors, such as the location and stage of the cancer, as well as the patient's symptoms. The treatment goal can range from aiming for a cure to controlling the symptoms and the progression of the cancer for as long as possible.

The standard therapy typically involves a combination of chemotherapy and immunotherapy. Chemotherapy is used to destroy cancer cells, while immunotherapy involves the use of antibodies that attach to cancer cells, marking them for destruction by the immune system. However, a drawback of these standard treatments is that they are not targeted and can also affect healthy cells, resulting in side effects like hair loss.

**How CGTs are Changing the Game?**
Cell and gene therapies (CGTs) are revolutionising the approach to DLBCL treatment. In some cases, patients may undergo high dose chemotherapy followed by a stem cell transplant. Autologous transplants involve using the patient's own stem cells collected prior to the procedure, while allogeneic transplants use stem cells from a donor. Stem cell transplantation can potentially address the underlying issue by replacing problematic cells and offering a potential cure.

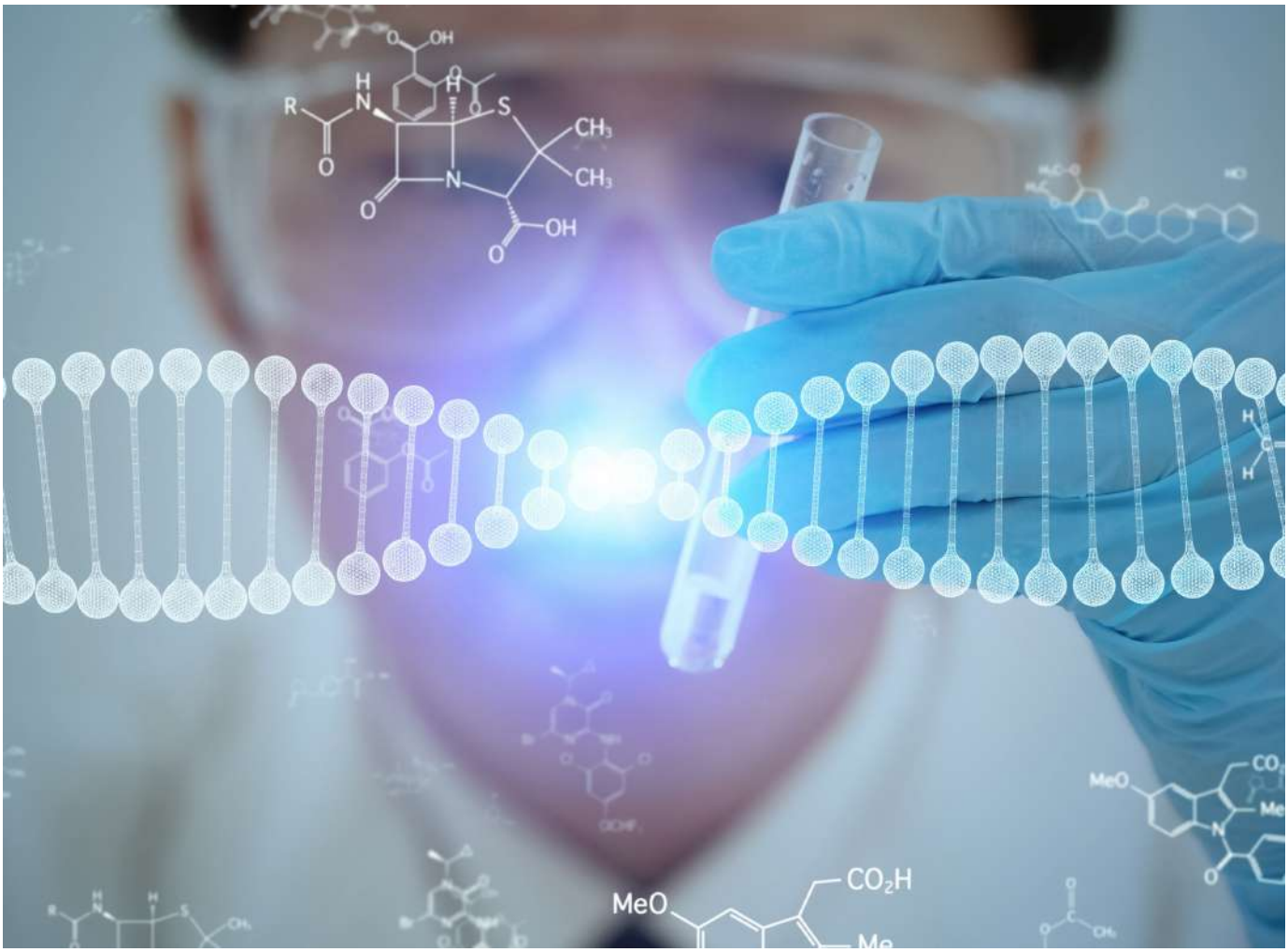
Another innovative therapy gaining prominence is CAR-T therapy, which is a personalized form of immune-cellbased treatment. CAR-T therapy involves modifying a patient's own immune cells to recognize and target cancer cells more effectively. This tailored approach has shown promising results in DLBCL and other types of cancer, providing new hope for patients.

CGTs are transforming the treatment landscape for DLBCL, offering targeted and potentially curative approaches that hold great promise for patients.

Gene therapy, specifically CAR-T therapy, has become available to patients with certain types of lymphoma. One notable example is Breyanzi, a CAR-T therapy that received approval from the U.S. FDA and the European Medicines Agency (EMA) in 2022 for the treatment of large B-cell lymphoma, including DLBCL.

To create each dose of Breyanzi, the patient's blood is extracted, and T-cells, a type of white blood cell crucial for the immune system, are collected. These T-cells are then modified in a laboratory to express chimeric antigen receptors (CARs) that allow them to recognize and bind to proteins on cancerous B-cells. This modification flags the cancer cells for elimination.

Clinical studies evaluating the effectiveness of Breyanzi have shown significant clinical benefits. Patients treated with Breyanzi demonstrated meaningful disease control, event-free survival, complete responses to therapy, and progression-free survival, especially in comparison to patients who experienced relapse within 12 months after standard first-line therapy. A high percentage of patients (86%) achieved either a complete or partial response, with 66% achieving a complete response.




While there are potential risks of side effects associated with Breyanzi therapy, the therapy has received approval because the benefits outweigh the risks. A single treatment with Breyanzi offers patients a more favourable prognosis compared to standard combination therapy with chemotherapy and immunotherapy.

In summary, Breyanzi has shown improved survival rates and, in some cases, the potential for curing patients. Its success has rapidly established CAR-T therapies as a valuable treatment option for patients with certain types of lymphoma.

Cell and gene therapies (CGTs) are shaping the future of oncology, bringing about a significant transformation in the field. The emergence of personalized medicine has challenged the traditional one-size-fits-all approach in healthcare, emphasizing the need for better health outcomes and patient-centric strategies. Consequently, CGTs have garnered substantial funding and support, revolutionizing the treatment landscape.

The potential of CGTs is evident in the FDA's approval of over 20 cell and gene therapy products. These innovative therapies have been authorized for the treatment of various chronic conditions, including cancers, mucogingival conditions, retinal dystrophy, and spinal muscular atrophy. This remarkable progress highlights the shift from disease management to seeking genuine, long-term cures facilitated by CGTs.

However, despite the promising prospects, several challenges lie ahead. The implementation of cell and gene therapies faces obstacles related to ethics, regulations, costs, and logistics. These factors could impede the translation of life-saving therapeutics from the research bench to the patient's bedside. Overcoming these hurdles is crucial to unlock the transformative potential of CGTs and extend their benefits to millions of individuals suffering from incurable chronic and rare diseases, thereby enhancing their quality of life.

In conclusion, CGTs represent the future of medicine, offering hope and transformative potential in treating conditions that previously had no effective treatments. While there are obstacles to overcome, the continued development and integration of cell and gene therapies have the potential to revolutionize the life science and pharmaceutical industries, paving the way for better outcomes and improved quality of life for patients. life-saving therapeutics from the bench to the bedside


## Richard Rossi

Richard is the Global Cold Chain & Strategic Projects Director at CRYOPDP. With over 20 years of experience in both diagnostic pathology, clinical trial pharmaceutical storage and logistics management, my industry knowledge allows me to apply best practices in everything that I do. From the creation of temperature-controlled warehouses for clinical trial product storage across Australia, Singapore and Japan, to launching 3PL pharma storage solutions in Australia, my history of leading and delivering state of the art pharma storage and supply lends itself to operational excellence.