Bringing Cell Therapies to Rare Disease Patients in Europe

Professor Boris Kramer MD. PhD

Introduction

Rare diseases are defined by sheer numbers of new patients per population. This definition is not helpful for the affected patient and his/her family. These conditions often lack effective treatment options due to their low prevalence. In addition, there are challenges in developing traditional pharmaceuticals for them.

The causes of rare diseases may be mostly of genetic origin. Cell therapy approaches offer potential solutions by targeting the underlying causes of these diseases at a cellular level. Therefore, 'repairing' the altered gene by gene therapy has been so successful in recent years for patients with genetic diseases.

Cell therapy is also known as cellular therapy or cytotherapy. It involves the use of living cells to treat various diseases and medical conditions. These cells can be sourced from various origins, including the patient's own cells (autologous), from a donor (allogeneic), or from cell lines.

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There are several types of cell therapies

Stem Cells

Stem cells can differentiate into various cell types in the body. They can be derived from sources such as bone marrow, adipose tissue, umbilical cord blood, or embryonic tissue.

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Mesenchymal Stem/Stromal Cells (MSCs)

Mesenchymal stem/stromal cells (MSCs) are stem cells found in various tissues, including bone marrow, adipose tissue, and umbilical cord. MSCs can differentiate into various mature cell types found in tissues, such as adipocytes (fat cells), chondrocytes (cartilage cells), and fibroblasts. This feature is called “multipotent’. MSC therapy is being explored for their immunomodulatory and regenerative properties.

Gene Therapy

While not strictly ‘cell’ therapy, gene therapy often involves modifying cells (typically using viral vectors) to introduce or correct the patient’s genetic material. This approach is used to treat genetic disorders or to enhance the therapeutic properties of cells used in other types of cell therapy.

The personalized nature of cell therapies allows for tailored treatments, which can be particularly beneficial for rare diseases characterized by genetic mutations or specific cellular dysfunctions.

This approach mandates not only the knowledge of the affected gene and the tools to replace the diseased gene copy in a tissue that is responsible for the disease but also that the ‘cured’ cells have a survival and/or selection benefit of some kind. Only with the proliferation and spread of healthy cells will the therapy have a lasting and sustainable success for the patient.

As research in this field advances, we expect to see more breakthroughs in treating rare diseases using cell therapy. The role of Partner Rare Limited in bringing potential therapies from development (‘bench’) to the patient (‘bedside’) starts in designing cell therapies, pre-clinical evaluation and characterization, clinical trials custom-made to meet regulatory requirements with EMA and FDA. Our dedicated team of experts provides personalized support throughout your development journey, ensuring your individual needs are addressed at every step.

About the Author

Professor Boris Kramer is a globally recognized expert in neonatology and translational medicine with over 25 years of experience. He has made significant contributions to developing innovative therapies for children and adults, particularly in cell therapies. His expertise spans preclinical research, clinical trial design, and regulatory affairs, leading to two European Orphan Designations for rare diseases and extensive experience in leading and sponsoring clinical trials.

As a Professor of Experimental Perinatology/Pediatrics, a prolific researcher (with over 302 peer-reviewed publications), and a dedicated mentor, Professor Kramer is passionate about advancing the field of medicine and improving patient outcomes.

To discuss your specific requirements or explore potential collaborations with Professor Kramer, contact Partner Rare for a complimentary consultation to discuss your specific needs.


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