OPEN ACCESS Freely available online

Advances in Pediatric Research

Perspective

Gene Therapy for Children: A Promising Frontier in Pediatric Medicine

Lucilla Nardo*

Department of Pediatrics, Grenoble Alpes University, Grenoble, France

DESCRIPTION

Gene therapy is an innovative and rapidly advancing field in medicine that holds significant potential for treating a variety of genetic disorders, some of which have long been considered incurable. In the context of pediatric care, gene therapy offers a new hope for children affected by inherited diseases, many of which are severe, life-limiting or debilitating. By addressing the root cause of certain genetic conditions at the molecular level, gene therapy has the potential to offer long-lasting or even permanent solutions, significantly improving the quality of life for affected children and their families.

Role of gene therapy

Gene therapy involves introducing, removing, or altering genetic material within a patient's cells to correct or replace defective genes responsible for disease. This can be done by directly delivering healthy copies of genes into cells, repairing mutated genes or replacing faulty genes with functional ones. The goal is to either correct the genetic defect or modify the expression of genes in a way that alleviates symptoms or stops the progression of disease.

For children, gene therapy has shown particular promise in treating inherited conditions such as cystic fibrosis, Duchenne muscular dystrophy, hemophilia and certain types of inherited blindness, among others. Many of these disorders are caused by single-gene mutations that disrupt normal function and traditional treatment options often provide only symptomatic relief rather than addressing the underlying cause.

Development of gene therapy

The development and use of gene therapy in children present unique challenges and considerations. First and foremost, safety is a major concern. As gene therapy is still a relatively new and evolving field, the potential risks, including immune reactions to the introduced genetic material, unintended genetic changes, or the development of malignancies, need to be thoroughly evaluated. Clinical trials in children are carefully designed to ensure that any risks are minimized and that the benefits outweigh the potential harms. Ethical considerations are also essential, particularly when it comes to changing the genetic material of minors who may not be able to provide informed consent themselves. In these cases, parental consent and rigorous oversight by ethical review boards are essential.

Another challenge in pediatric gene therapy is the delivery of genetic material to the right cells in the body. Children's bodies can react differently to treatments than adult bodies, and they may require designer methods of gene delivery that take into account their age, size and developing immune systems. Various techniques are used for gene delivery, including viral vectors (modified viruses that carry the new genetic material), lipid nanoparticles and other delivery systems that are designed to effectively transport the therapeutic gene to the target cells. In the case of certain diseases, such as those affecting the eyes, injections of the therapeutic gene directly into the tissue may be used.

CONCLUSION

Gene therapy represents a transformative approach to treating genetic disorders in children. Likewise, gene therapies for hemophilia, a blood clotting disorder, have shown promise in reducing or even eliminating the need for regular blood clotting factor infusions, a treatment that many children with hemophilia must undergo throughout their lives. While challenges remain, the progress made in the field is paving the way for more effective, targeted, and potentially curative treatments for pediatric patients. As study continues and therapies become more refined, gene therapy has the potential to revolutionize the treatment of genetic diseases and significantly improve the lives of children worldwide.

Correspondence to: Lucilla Nardo, Department of Pediatrics, Grenoble Alpes University, Grenoble, France, Email: luci.nd@gau.fr

Received: 24-Feb-2025, Manuscript No. LDAPR-25-37323; **Editor assigned:** 26-Feb-2025, PreQC No. LDAPR-25-37323 (PQ); **Reviewed:** 12-Mar-2025, QC No. LDAPR-25-37323; **Revised:** 19-Mar-2025, Manuscript No. LDAPR-25-37323 (R); **Published:** 26-Mar-2025, DOI: 10.35248/2385-4529.24.12.101

Citation: Nardo L (2025). Gene Therapy for Children: A Promising Frontier in Pediatric Medicine. Adv Pediatr Res. 12:101.