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Beyond Factor Replacement: Gene Therapy As a Paradigm Shift in Haemophilia Management

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ABSTRACT

This review examines recent developments in Haemophilia treatment, with a focus on gene therapy approaches. Haemophilia A and B are genetic disorders caused by deficiencies in coagulation factors VIII and IX, respectively. While current treatments rely on regular infusions of clotting factors, they face limitations including frequent administration and the risk of inhibitor development. Gene therapy emerges as a promising alternative, offering the potential for long-term therapeutic gene expression. The review analyzes various gene therapy strategies, with particular attention to adeno-associated virus (AAV) vector-mediated approaches. Recent clinical trials have shown encouraging results in both the Haemophilia types, demonstrating sustained factor expression and reduced bleeding episodes in some patients.

The paper discusses the complexities inherent in gene therapy, including immune responses, vector optimization, and safety considerations. It also explores genome editing technologies as potential future treatments. Additionally, the review touches on the economic implications of gene therapies, considering aspects of cost-effectiveness and health technology assessment.

While gene therapy shows promise, challenges remain in ensuring long-term efficacy and safety. Ongoing research aims to address these issues, potentially transforming Haemophilia treatment from chronic management to a one-time curative approach. The review concludes by highlighting areas for further investigation and the potential impact of these therapies on Haemophilia care.

Keywords: adeno-associated virus, gene therapy, Haemophilia, lentivirus, Factor VIII, Factor IX

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