Gene Therapy Concepts in the Treatment of Haemophilia

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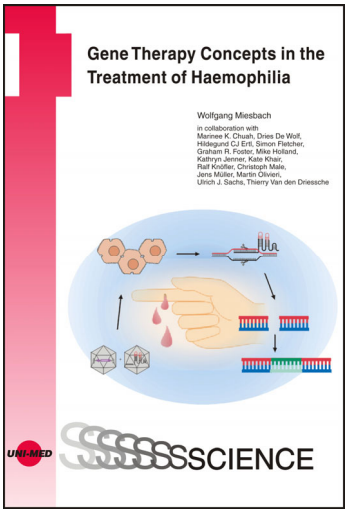

Over the past decade, gene therapy has emerged as a groundbreaking new treatment option, particularly with the approval of products for haemophilia A and B. Thanks to the use of adeno-associated viruses (AAV) as vectors for the delivery of a functional gene to the body, these novel treatments promise a high degree of liberation from burdensome regular replacement therapy with coagulation factors.

This book presents the current knowledge on the potential of these new gene therapies in the treatment of haemophilia A and B. It covers the fundamentals of haemophilia as well as the various gene therapy approaches (AAV, lentivirus, gene editing), and includes specific aspects such as the selection of suitable patients for gene therapy, the effects on coagulation factor levels, liver-related issues, and the immune response to AAV-mediated gene therapy. The necessity of interdisciplinary collaboration and the innovative "hub and spoke" model are discussed.

This book is intended to serve as a bridge, closing the gap between current knowledge and future developments, and it aims to be a comprehensive source of information for professionals in both clinical practice and research.

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