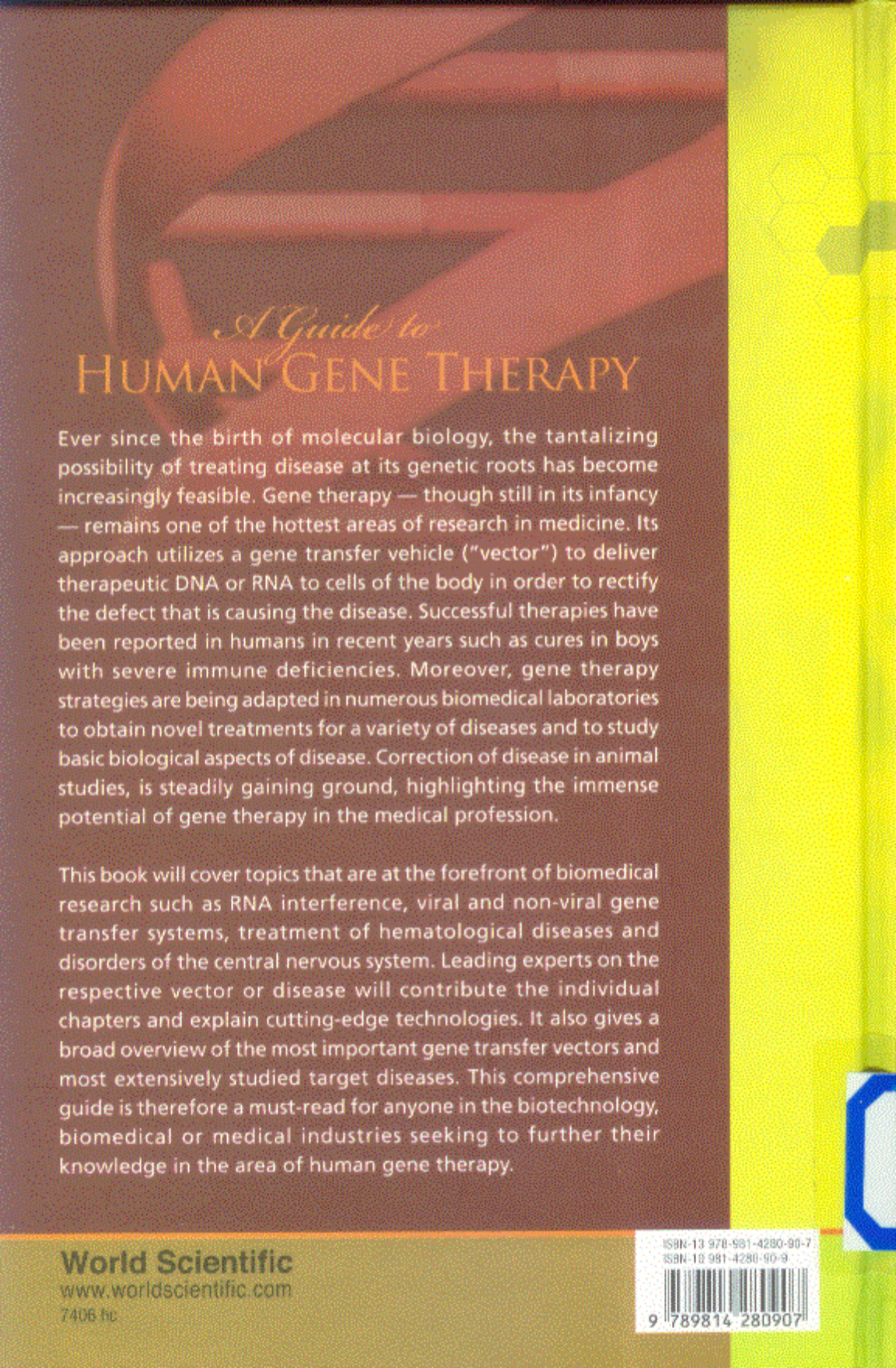




A Guide to **HUMAN GENE THERAPY**

Ever since the birth of molecular biology, the tantalizing possibility of treating disease at its genetic roots has become increasingly feasible. Gene therapy — though still in its infancy — remains one of the hottest areas of research in medicine. Its approach utilizes a gene transfer vehicle ("vector") to deliver therapeutic DNA or RNA to cells of the body in order to rectify the defect that is causing the disease. Successful therapies have been reported in humans in recent years such as cures in boys with severe immune deficiencies. Moreover, gene therapy strategies are being adapted in numerous biomedical laboratories to obtain novel treatments for a variety of diseases and to study basic biological aspects of disease. Correction of disease in animal studies, is steadily gaining ground, highlighting the immense potential of gene therapy in the medical profession.

This book will cover topics that are at the forefront of biomedical research such as RNA interference, viral and non-viral gene transfer systems, treatment of hematological diseases and disorders of the central nervous system. Leading experts on the respective vector or disease will contribute the individual chapters and explain cutting-edge technologies. It also gives a broad overview of the most important gene transfer vectors and most extensively studied target diseases. This comprehensive guide is therefore a must-read for anyone in the biotechnology, biomedical or medical industries seeking to further their knowledge in the area of human gene therapy.

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