The emerging new field of prenatal gene therapy is based on the rapid scientific and technical advances in fetal medicine, molecular biology, and gene therapy over the last two decades. This novel and still preclinical research subject aims at applying gene therapy during pregnancy for the prevention of human diseases caused by early onset congenital or gestation-related conditions. The present volume summarizes the accumulated scientific knowledge and practical experience over more than 15 years of research by leading scientists in the fields of gene therapy, fetal medicine, and medical ethics. It provides a unique and comprehensive overview of the concept of prenatal gene therapy, its potential target diseases, its advantages and possible adverse effects, and of the ethical and societal implications of this approach. This book contains detailed protocols for vector production, for breeding and husbandry of the animal models, for the surgical procedures of gene delivery in large and small animals, and for the methods of gene transfer analysis. The various chapters are introduced by overviews covering the different vector systems, animal models, and analysis methods used in basic research on prenatal gene therapy, and in preparation for human application. Although prenatal disease is the main target of application in this volume, the chapters on vector generation, production, and testing compiled here provide detailed state-of-the-art knowledge useful for other gene therapy projects beyond the scope of fetal medicine.

Written for: Gene therapists, obstetricians, specialists in perinatal medicine, human geneticists, molecular biologists, medical ethicists.

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