Gene Therapy of Cancer
Methods and Protocols

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Since the discovery of the molecular structure of genes and the unveiling of the molecular basis of numerous human diseases, scientists have been fascinated with the possibility of treating certain diseases by transducing foreign DNA into the affected cells. Initially, it was proposed that the foreign DNA could either replace defective nonfunctional genes, or code for therapeutic proteins. This concept has evolved into the rapidly growing field of gene therapy.

Even though surgery, radiotherapy, and chemotherapy are widely available and routinely used for cancer treatment, these therapies fail to cure approximately 50 percent of cancer patients. Therefore, since it is a disease characterized by aberrant gene expression, cancer has been a target of gene therapy research since the inception of this treatment modality. Numerous cancer gene therapy strategies are currently being investigated, including gene replacement therapy, the regulation of gene expression to modulate immunological responses to tumors, the direct killing of tumor cells, and direct interference with tumor growth. In this context, gene transfer systems, tumor-specific expression vectors, and novel therapeutic genes have been extensively studied. All these strategies aim for the selective destruction of human malignant disease while circumventing the destruction of nonmalignant cells and tissues thereby minimizing toxicity to the patient.

Rapid progress in the field of cancer gene therapy, exemplified by the vast number of publications in this area, creates a challenging situation for scientists and clinicians who need to be cognizant of the most recent advances in gene transfer techniques. This volume of *Gene Therapy of Cancer: Methods and Protocols* in the *Methods in Molecular Medicine* series will provide researchers with a broad array of methods used to study cancer gene therapy in both the laboratory and clinical trials. Moreover, several chapters are included to provide short overviews of specialized gene therapy strategies for the treatment of particular malignancies.

*Gene Therapy of Cancer: Methods and Protocols* does not provide comprehensive reviews of all methodologies currently used for gene therapy of cancer. Rather the topics we have selected consist of approved procedures,
current trends, and representative strategies in cancer gene therapy using different classes of therapeutic genes, suppressor genes, antisense oligonucleotides, ribozymes, viral- and nonviral-vector systems, and tumor targeting approaches at the preclinical and, more importantly, at the clinical level. For cancer gene therapy to be successful in the treatment of human cancers, extensive preclinical evaluation is essential. Therefore, the first part of this book discusses relevant experiments from preclinical studies followed by clinical gene therapy protocols in the second part.

*Gene Therapy of Cancer: Methods and Protocols* should provide practical guidance for basic and clinical researchers, as well as graduate and postgraduate students working in the exciting and emerging field of gene therapy.

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