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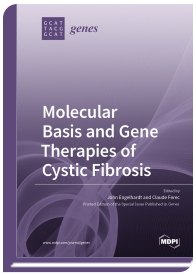
Special Issue Reprint

Molecular Basis and Gene Therapies of Cystic Fibrosis



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Summary of Genes. Thirty years ago, the gene responsible for cystic fibrosis (CF), a recessive genetic disease caused by mutations in the cystic fibrosis transmembrane conductance regulator gene, was identified. This progress has considerably changed our understanding of the pathophysiology of CF and has paved the way for the development of novel and specific therapies for the disease. The CFTR gene contains 27 exons and is characterized by a frequent three base pair deletion of the p.Phe508del. As a result of collaborative work, today more than 2000 mutations have been reported in the gene, and their impact on protein function is now more evident and useful in designing new strategies to correct the gene defect. The field of gene therapy, as illustrated by Ziying Yan in this book, has worked on identifying an efficient vector system for the delivery of the wild-type CFTR gene to the lung. At the same time, animal models have been developed in mice, rats, rabbits, zebrafish, ferrets, and pigs to establish the efficacy of gene delivery. These animals are also of the utmost importance in testing new molecules as modulators or correctors to improve the CFTR lung function. During the last three decades, the epidemiology of CF has dramatically changed, as today cystic fibrosis is now a chronic adult pulmonary disease.

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