Treatment of Cystic Fibrosis and Other Rare Lung Diseases (Milestones in Drug Therapy)

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This volume describes the pathogenesis and pathophysiology of several pulmonary diseases as well as their treatment. It also discusses the underlying genetic and molecular biological basis, which opens the way for new treatments for these conditions.

It focuses on the treatment of cystic fibrosis including CFTR (cystic fibrosis transmembrane-conductance regulator) modulator therapies, drug therapies that augment airway surface liquid as well as anti-inflammatory and anti-infective therapies. Further topics include long-term, low-dose macrolide therapy for diffuse panbronchiolitis; novel agents for previously untreatable idiopathic pulmonary fibrosis; possible new treatments for pulmonary alveolar proteinosis (PAP); and multiple novel therapeutic targets for treating lymphangiomyomatosis. Research into these conditions has led to major advances in our understanding of the underlying genetic and molecular basis of this disease, and to dramatic improvements in survival and quality of life for affected individuals.

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