Mutation-specific therapies in cystic fibrosis

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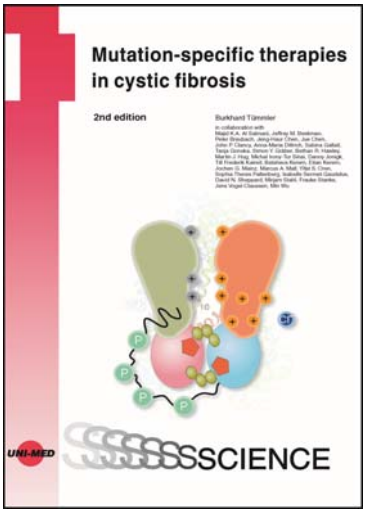

Cystic fibrosis is a severe ion channel disease of autosomal recessive inheritance that is caused by mutations in the Cystic Fibrosis Transmembrane Conductance Regulator (CFTR) gene. Thanks to continuously improved symptomatic treatment during the last five decades this lethal paediatric disease has been transformed into a chronic disorder with a median life expectancy of nowadays more than 50 years.
This 2nd edition provides the reader with the background and on-going preclinical and clinical research for the development of mutation-type specific therapy of cystic fibrosis. Starting with the biology and biomarkers of CFTR in the context of cystic fibrosis, the reader gets insight into the basic and clinical research of CFTR modulators from bench to bedside. A large section of the book focuses on the clinical trials, post-approval observational studies and the real-world experience with the CFTR modulators.

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