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Mutation-specific therapies in cystic fibrosis - Current status and prospects

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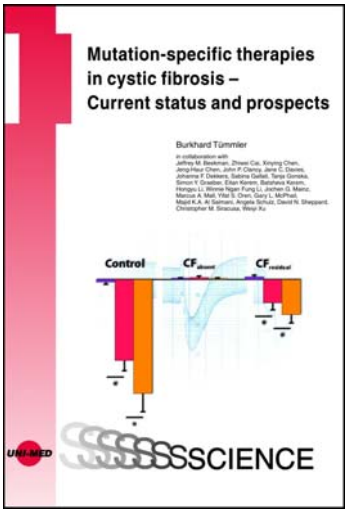

Personalized medicine promises that medical decisions, practices and products are tailored to the individual patient. Cystic fibrosis, an inherited disorder of ion transport in exocrine glands, is the first successful example of customized drug development for mutation-specific therapy. This monograph reports on the contributions of the basic and clinical sciences to translate knowledge of the basic defect of cystic fibrosis into a medical product. Starting from the molecular pathology with focus on the affected ion channel CFTR, the bioassays to assess CFTR function in humans are introduced followed by a state-of-the-art report of preclinical and clinical research on CFTR modifying drugs. Patients and physicians describe their experience of therapy with ivacaftor, the first CFTR modulator approved for human use, and the final chapter looks ahead for the next steps of cystic fibrosis therapeutics development.

Klinische Lehrbuchreihe
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