Biomedical Literature Q&A with Generative AI

Medical Literature: Cystic Fibrosis and CFTR Protein Targeting Drugs

Introduction:

Cystic fibrosis (CF) is a genetic disorder caused by mutations in the CFTR (Cystic Fibrosis Transmembran

This gene encodes a protein that functions as a chloride channel, regulating the movement of ions across

Mutations in the CFTR gene disrupt this process, leading to thick mucus buildup in organs such as the lun

Treatment Advances:

Recent advances in medicine have led to the development of drugs targeting the defective CFTR protein.

These drugs aim to improve chloride ion transport and alleviate symptoms.

CFTR Modulators:

1. **Ivacaftor (Kalydeco)**: A CFTR potentiator that enhances the gating function of CFTR proteins with sp

2. **Lumacaftor/Ivacaftor (Orkambi)**: A combination therapy that works for patients with the F508del muta

3. **Tezacaftor/Ivacaftor (Symdeko)**: Similar to Orkambi but with fewer side effects, designed for F508del

4. **Elexacaftor/Tezacaftor/Ivacaftor (Trikafta)**: A triple combination therapy effective in patients with at le

Emerging Therapies:

- Gene therapy approaches aim to correct the underlying genetic defect in CF.

- mRNA-based treatments are being explored to produce functional CFTR proteins in affected cells.

Conclusion:

Advancements in CFTR modulator therapies have transformed cystic fibrosis from a fatal pediatric disease

Ongoing research continues to explore novel therapies to target rare CFTR mutations and further improve

References:

1. National Institute of Health (NIH): Overview of CFTR Modulators

2. PubMed Central: Advances in Cystic Fibrosis Treatments

3. Cystic Fibrosis Foundation: Drug Development Pipeline

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