**Title:**

Treatment decision-making for using CFTR modulator therapy in patients with cystic fibrosis

**Lay Title:**

How doctors decide to prescribe CFTR modulator therapies to their patients

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**What was your research question?**

There are 5 approved CFTR modulator therapies to treat people with cystic fibrosis (CF). Despite established safety and clinical benefits, there is variability in how these treatments are prescribed by CF doctors. We sought to understand the factors doctors consider when prescribing CFTR modulators to their patients with CF.

**Why is this important?**

The development and approval of CFTR modulator therapies has changed the way CF is treated. CFTR modulators treat the underlying cause of CF disease, improving lung function and CF symptoms. However, it is important to understand how doctors make decisions to prescribe a specific CFTR modulator for a given patient.

**What did you do?**

We developed 20 different patient profiles (called clinical vignettes) that described 10 hypothetical pediatric patients and 10 hypothetical adult patients with CF who were eligible for CFTR modulator therapy. Important information for prescribing a CFTR modulator, including patient genotype, health status, age, pancreatic sufficiency status, and any current medications, were included in each vignette. Eighty pediatric and adult CF doctors across five Europe countries took an online treatment decision exercise where they were asked to decide on a treatment for each patient profile and provide a reason for their treatment decision.

**What did you find?**

There was variability in how CFTR modulator therapies were prescribed by doctors, even when presented with the same patient profile. This variability was largely explained by doctor characteristics, including behaviors, attitudes, and prior experiences, rather than specific individual disease factors. Doctors who described themselves as being proactive and pushing boundaries or who previously prescribed a CFTR modulator were more likely to prescribe a CFTR modulator. We found that being 12 years of age or younger or pancreatic sufficient decreased the probability of being prescribed a CFTR modulator. Of the four CFTR modulators available at the time of this study, elexacaftor/tezacaftor/ivacaftor was the most frequently prescribed regimen but was not a universal choice among doctors.

**What does this mean and reasons for caution?**

Our findings confirm that there is variability among doctors in how they reach the decision to prescribe a CFTR modulator and that this is primarily due to their own behavioral characteristics and confidence based on prior experience. Therefore, it is important for doctors to have as much knowledge as possible on the use, safety, and effectiveness of CFTR modulators to help inform treatment decisions when it comes to possibly prescribing a CFTR modulator for their patients with CF.

**What’s next?**

As CFTR modulator use continues to expand, and more real-world clinical evidence emerges, we will likely see further changes in doctors’ prescribing habits. This approach could be employed in future studies to understand how CFTR modulator prescribing patterns evolve over time or even applied to treatments for other conditions.

**Original manuscript citation in PubMed**

<https://pubmed.ncbi.nlm.nih.gov/40713199/>