

# Cystic Fibrosis Research News

**Title:**

WHAT IS THE IMPACT OF INTERRUPTION OF CFTR MODULATOR THERAPIES IN CHILDREN WITH CYSTIC FIBROSIS?

**Lay Title:**

Modulatory therapy discontinuation of children with cystic fibrosis

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**What was your research question?**

In order to determine how important it is to continue modulator treatment, we tried to assess the benefits of them as well as the effects of a subsequent therapy interruptions.

**Why is this important?**

Patients in low-middle income nations are particularly vulnerable due to the prohibitive expense of modulator therapies. In some countries, social insurance does not pay for them because of their prohibitive costs. In Türkiye, families whose children were eligible for modulators began suing the social security system to be able to access these drugs. Some families successfully brought the matter to court and obtained access to these therapies. However, after initiating their drugs, some patients had supply interruption because of lawyer fees and the problems experienced in following the legal procedures.

**What did you do?**

We looked at patients with CF, age under 18 years and followed at Hacettepe University Pediatric Pulmonology Department who had been treated with CFTR modulators but whose access was interrupted during the period between August 2021 and October 2022. We compared pulmonary function test, body mass index, body weight, disease specific health



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related quality of life measure between 'before modulator treatment', 'during treatment' and 'after interruption of treatment' periods.

## **What did you find?**

Our results show that when therapy is stopped, there is a significant loss of the therapeutic benefit of modulators, with several measures showing a dramatic decline. In many individuals, the decline in lung functions, body weight and body mass index, was lower than it was before modulators were started, and the results were worse in patients with *Pseudomonas aeruginosa* in the airways.

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

The benefit of modulators is lost when the therapy is discontinued, and this effect appears to occur quickly. Patients with cystic fibrosis who are eligible, should globally have equal access to modulators and processes should be in place to assure continued access to the treatment.

## **What's next?**

Steps should be implemented to provide global equitable access to these treatments. Thankfully, a global advocacy movement for equal access to affordable CF medicines is expanding.

## **Original manuscript citation in PubMed**

<https://pubmed.ncbi.nlm.nih.gov/38762388/>