# Cystic Fibrosis Research News

**Title:**

COST-EFFECTIVENESS ANALYSIS OF GENETIC TOOLS TO PREDICT TREATMENT RESPONSE IN PATIENTS WITH CYSTIC FIBROSIS

**Lay Title**:

Is it cost-effective to use genetic tools to predict how well patients with cystic fibrosis will respond to treatment?


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**What was your research question?**

Researchers set out to develop tools that could predict how well individuals with cystic fibrosis (CF) would respond to novel drugs for CF. However, testing with these tools comes with additional expenses.

Whether upfront testing with predictive tools to guide the prescription of novel drugs is worth the extra cost?

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## Why is this important?

CF is a genetic disease affecting more than 4,000 people in Canada. It can cause frequent lung infections, breathing and digestive problems and affect life expectancy.

Recently, new drugs, namely elexacaftor-ivacaftor-tezacaftor (ETI) have become available that address the genetic defect that causes CF, but not all patients respond to these drugs. As these drugs may cost over $300,000 per year and may have side effects, there is a need for reliable tools to predict who will respond well to these drugs and who will not. Therefore, it is important to evaluate both the benefits and the costs of these testing tools.

## What did you do?

We compared two strategies using a mathematical model that mimicked the progression of CF in a population similar to CF population of Canada. The first strategy was to give ETI to all patients in addition to standard of care (SoC), while the second strategy involved pre-treatment testing by predictive tools. Patients, who tested positive would receive ETI plus SoC and those who tested negative would receive SoC only.

We compared the costs and health benefits associated with each strategy.

## What did you find?

We found that giving ETI to all patients, in addition to standard care, could improve the quality and length of life for people with CF. However, using this medication for everyone will substantially increase healthcare costs. People who received ETI were less likely to have severe complications of CF and need certain medical procedures, but the savings from these benefits did not make up for the increased medication costs.

We found that it is more cost-effective to test people first to see if they would benefit from ETI, rather than giving ETI to everyone without testing.

## What does this mean and reasons for caution?

Currently, clinicians lack tools to determine the best course of treatment for each patient.

Since no test is perfect, there would always be patients who might have benefited from treatment but incorrectly identified as non-responders. As these tools become more accurate, more patients that are responsive would be correctly identified and treated. Meanwhile, non-responders would not receive unnecessary intervention, which would reduce cost burden.

Our study suggests that identifying eligible patients through pre-treatment predictive testing could maximize health benefits and reduce economic burden.



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**What's next?**

Our results are timely and will help to ensure that patients who would likely to benefit are given access to this regimen. This research has important implications for CF care, insurance coverage and reimbursement policies, especially in areas with limited resources.

**Original manuscript citation in PubMed**

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