

Cystic Fibrosis Research News

Title:

Projecting the impact of delayed access to elexacaftor/tezacaftor/ivacaftor for people with Cystic Fibrosis

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What was your research question?

How will elexacaftor/tezacaftor/ivacaftor (Trikafta™) impact the health care needs of people living with CF in the future? If Trikafta™ is available now, how many hospitalizations and deaths will be avoided by 2030? What will happen if there is a delay in (Trikafta™) approval?

Why is this important?

Elexacaftor/tezacaftor/ivacaftor (Trikafta™) is a new treatment for CF that corrects the underlying protein defect. In clinical trials it improved lung function and reduced hospitalizations for chest infections. More than 90% of people with CF may benefit from Trikafta, and this new therapy could profoundly change the characteristics and health care needs of people living with CF. At the moment Trikafta™ is not available in many countries; it is not available in Canada. Forecasting the impact of Trikafta™ for people living with CF can provide a deeper understanding of their long-term needs and anticipate the demand for future services and healthcare resources.

What did you do?

Our study used a technique called microsimulation to predict what might happen to Canadians living with CF in the year 2030. We used the Canadian CF Registry data which is a comprehensive database of nearly all Canadians with CF. The forecasting models looked at three key scenarios: (1) if Trikafta™ was not available in Canada (2) if Trikafta™ was available in 2021 (early) and (3) if access to Trikafta™ was delayed until 2025 (delayed).



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What did you find?

Our results show that early introduction of Trikafta™ would result in 60% fewer people with severe lung disease, 18% increase in people with mild lung disease and 19% fewer hospitalizations or home intravenous courses for chest infections by 2030 compared to no drug available. If access to Trikafta™ is delayed, the improvements in health will be substantially reduced across all outcomes therefore, delayed access to this effective medication will result in preventable health care costs and death.

What does this mean and reasons for caution?

Forecasting models are not meant to be interpreted literally to predict the future. This research provides a summary of the likely impact of new therapies in contrast to the current trends. We made several assumptions about the expected improvements in lung function and chest infections, and our results are affected by these assumptions.

What's next?

As new information becomes available these forecasts will need to be updated to better reflect current realities.

Original manuscript citation in PubMed

<https://pubmed.ncbi.nlm.nih.gov/32855088/>