



Cystic Fibrosis Research News

Title:

Factors associated with prescription of elexacaftor/tezacaftor/ivacaftor among people with cystic fibrosis aged 12 years or older with at least one F508del allele

Lay Title:

Understanding rates of and factors associated with elexacaftor/tezacaftor/ivacaftor (ETI) prescription

Authors:

Georgene E Hergenroeder¹, Jonathan V Todd², Josh S. Ostrenga², Christopher H Goss³, Raksha Jain⁴, Wayne Morgan⁵, Gregory S. Sawicki⁶, Michael S Schechter⁷, Elizabeth A Cromwell², Clement L Ren¹

Affiliations:

1. Children's Hospital of Philadelphia, Philadelphia, PA
2. Cystic Fibrosis Foundation, Bethesda, MD
3. University of Washington, Seattle, WA
4. University of Texas Southwestern Medical Center, Dallas, TX
5. The University of Arizona, Tucson, AZ
6. Boston Children's Hospital, Boston, MA
7. Children's Hospital of Richmond at Virginia Commonwealth University, Richmond, VA

What was your research question?

What characteristics, such as age, lung function, or co-existing conditions (if any), are associated with prescription of ETI?

Why is this important?

We have learned from the CF Foundation Patient Registry (CFFPR) that 87.9% of eligible people with CF (PwCF) were prescribed a CFTR modulator in 2020. ETI has led to tremendous improvements in lung function and quality of life for people with CF (PwCF), and we were interested in determining whether there are characteristics associated the time to being prescribed ETI. This knowledge is important to ensure consistency of care and that all eligible PwCF have an equal opportunity to access ETI.

Cystic Fibrosis Research News

What did you do?

We used the CF Foundation Patient Registry to compare PwCF who were eligible for ETI and prescribed ETI with PwCF who were eligible for ETI but not prescribed ETI. Among PwCF prescribed ETI, we performed additional statistical analyses to compare how long it took to start ETI among different groups and look for risk factors associated with timing of first ETI prescription.

What did you find?

We found that 91% of people eligible for ETI were prescribed ETI within 3 years of approval. People prescribed ETI were younger, had lower lung function, more pulmonary exacerbations in the prior year, earlier age of diagnosis, and were more likely to have been prescribed another CFTR modulator previously (if eligible). Public health insurance, higher lung function, Black race and Hispanic ethnicity were associated with lower hazards (e.g., later) of ETI prescription whereas prior modulator prescription, pancreatic insufficiency, increased exacerbation frequency and prior infections were associated with a higher hazard (earlier) of prescription.

What does this mean and reasons for caution?

These findings mean that the timing of ETI prescription was associated with both severity of CF lung disease and demographic factors. Longer time to ETI prescription among Black and Hispanic PwCF highlights racial and ethnic disparities, and further work is critical to identify strategies to ensure that all eligible PwCF are benefiting from ETI.

Our study only looked at PwCF who were age 12 and older and who had at least one copy of the most common CF-causing variant, F508del. Therefore, our results may not be applicable to younger people or people with other CF-causing variants.

What's next?

In the next stage of our research, we will be looking at characteristics associated with larger or smaller changes in lung function and larger or smaller changes in body mass index for PwCF who start ETI.

Original manuscript citation in PubMed

<https://pubmed.ncbi.nlm.nih.gov/39472230/>