



Cystic Fibrosis Research News

Title:

Rate and Predictors of Prescription of Lumacaftor – Ivacaftor in the 18 Months Following Approval in the United States

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What was your research question?

What were the characteristics of people with cystic fibrosis (CF) with two copies of the F508del mutation who were prescribed lumacaftor-ivacaftor following FDA approval for those 12 years and older in 2015 and those 6-11 years old in 2016?

Why is this important?

CFTR modulators are the first group of therapies developed to target the basic protein defect in CF. Nearly half of people with CF have two copies of the F508del mutation and may benefit from lumacaftor-ivacaftor therapy. Post-approval lumacaftor-ivacaftor prescription may vary based on clinician, health system, and patient factors.

What did you do?

After identifying people who were eligible for treatment with lumacaftor-ivacaftor from the CF Foundation Patient Registry, we excluded those with organ transplants or who had already received lumacaftor-ivacaftor therapy in clinical trials. We then looked at three comparisons in over 10 500 people: 1) patients 12 years and older with a reported lumacaftor-ivacaftor prescription compared to those without a prescription; 2) for those with a prescription, which patients received this within six months of FDA approval compared to the following year; 3) younger patients (6 to 12 years) with a reported prescription compared to those without. We looked at whether there were differences among patients based on insurance type, US region of residence, other CF medications, and CF care center size.

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What did you find?

Prescription rate was lower for lumacaftor-ivacaftor than ivacaftor, especially in younger children. About half (53%) of eligible patients had reported prescriptions, with clinical, social, regional, and health insurance differences. Predictors of prescriptions for both younger and older children included insurance type, US residential region, azithromycin/ mucolytic prescriptions, and attend smaller CF centers. Older children with prescriptions within six months of approval appeared to have more severe disease, more previous clinical trial participation, attend pediatric/affiliate programs, and have inhaled antibiotics prescribed. Younger children with prescriptions were more likely to also have azithromycin and dornase alfa prescribed, and private insurance.

What does this mean and reasons for caution?

The Patient Registry does not track whether prescriptions are filled or taken, so it is unknown whether patients took lumacaftor-ivacaftor as prescribed. The high cost of CFTR modulator therapy may affect prescribing patterns or access based on health insurance. Documentation errors and missing information in the Patient Registry, although generally uncommon, may lead to under-estimation of this medication.

What's next?

Patient registries are important data sources for further exploring long-term, real-world use of CFTR modulator therapies. Further study of the long-term safety and effectiveness of lumacaftor-ivacaftor therapy may address or add to concerns of clinicians and people with CF who are eligible for these medications.

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