Journal of Cystic Fibrosis
The Official Journal of the European Cystic Fibrosis Society

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# Cystic Fibrosis Research News

**Citation:**

Sawicki GS, Dasenbrook E, Fink AK, Schechter MS. Rate of Uptake of Ivacaftor Use after U.S. Food and Drug Administration Approval among Patients Enrolled in the U.S. Cystic Fibrosis Foundation Patient Registry. Annals of the American Thoracic Society. 2015 Aug;12(8):1146-52. PMID: 26073026.

**What was your research question? (50 words maximum)**

We wanted to examine the rate of uptake and patterns of documented ivacaftor use among U.S. patients with cystic fibrosis (CF) during the first year after approval. We also wanted to compare eligible patients with and without reported ivacaftor use and describe characteristics of early adopters of ivacaftor use.

**Why is this important? (100 words maximum)**

Continuous CF therapies have different rates of prescribed use, and treatments are rarely prescribed to more than 80% of eligible patients. In January 2012, ivacaftor was approved by the U.S. Food and Drug Administration (FDA) for individuals with CF ages 6 and older with at least one copy of the G551D mutation. In clinical trials, ivacaftor treatment showed significant improvements in multiple clinical outcomes over a 1-year period. Understanding more about ivacaftor will allow us to learn more about its impact for people living with CF.

**What did you do? (100 words maximum)**

We looked at a group of patients in the U.S. Cystic Fibrosis Foundation Patient Registry (CFFPR) who used ivacaftor in 2012. Ivacaftor-eligible patients were defined as any person age 6 or older with a G551D mutation. We performed statistical analyses and separated data by age group to compare clinical and general characteristics of eligible patients with and without recorded ivacaftor

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use in 2012. We also looked at early and later ivacaftor users in 2012. Early is defined as starting ivacaftor from February to July, and late as starting from July to December.

**What did you find? (100 words maximum)**

In 2012, there were 1,087 people with CF who had G551D mutations in the CFFPR. By June 2012, 64% of eligible patients had recorded use of ivacaftor. This number increased to 81% by the end of 2012. For eligible patients aged 17 and under, 85% were prescribed ivacaftor. For eligible patients aged 18 and older, 79% were prescribed ivacaftor. Recorded ivacaftor prescriptions were different by state. A decreased likelihood of ivacaftor use in adults with less than 4 clinic encounters in 2012 was the only association found with early use of ivacaftor.

**What does this mean and reasons for caution? (100 words maximum)**

Initiation of ivacaftor among eligible patients in the U.S. was very quick. Most ivacaftor use began within 4 months of FDA approval. Differences in prescriptions appeared related to patient age, older age of diagnosis, and less frequent clinical encounters. There are some reasons for caution with these findings, including: no way of knowing if patients filled the prescription or regularly took the medication, and not being able to evaluate factors other than clinical and general characteristics that may be related to starting a new treatment.

**What's next? (50 words maximum)**

Only a small percentage of eligible patients were not receiving ivacaftor within a few months of its availability. Therefore, future evaluations of ivacaftor effectiveness will require researchers to find appropriate groups, such as those

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included in label expansions, to compare. Registries are valuable tools for longer-term analyses on effectiveness.