



Cystic Fibrosis Research News

Citation:

Feng LB, Grosse SD, Green RF, Fink AK, Sawicki GS. Precision Medicine in Action: The Impact of Ivacaftor on Cystic Fibrosis-Related Hospitalizations. Health Affairs. 2018 May;37(5):773-779. PMID: PMC6038921.

What was your research question? (50 words maximum)

We wanted to assess the impact of the drug ivacaftor (brand name Kalydeco) on cystic fibrosis-related hospital stays. This analysis is a continuation of research done by other scientists. Our goal was to find evidence that supported or challenged results stating that ivacaftor improves health outcomes and decreases hospital stays.

Why is this important? (100 words maximum)

Cystic fibrosis (CF) is a life-threatening genetic disease. Ivacaftor is the first drug to target CF at a cellular level instead of just treating symptoms. Ivacaftor is a great example of a precision medicine which seeks to gain better health results for people by treating their unique genetic features. It is important for patients, doctors, and those responsible for the cost of drugs to understand ivacaftor's benefit as a precision medicine to provide better access to more patients.

What did you do? (100 words maximum)

We looked at data from a commercial research database over a seven-year period, from 2010 to 2016, from eligible patients with CF who were prescribed ivacaftor. We separated out analysis into two periods: pre-ivacaftor and post-ivacaftor. We counted hospital stays overall versus hospital stays with CF as the primary reason for hospitalization in these two periods. We also analysed two subgroups, for people who started ivacaftor under the initial FDA label and people who started under the later expanded label.

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What did you find? (100 words maximum)

The total sample included 143 people who had filled prescriptions for ivacaftor. Overall, the rate of hospital admissions decreased by over half from the period before filling the prescription to the period after filling. Decreases were similar in both adults and children. Admissions to the hospital related to CF decreased even more than all-cause admissions. Use of ivacaftor was also associated with 60 percent lower per person inpatient spending in the year following starting ivacaftor compared to the year before.

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

Our findings suggest that sustained use of ivacaftor can significantly reduce overall hospital stays, especially those related to CF complications. There are some reasons for caution with our findings which include: not being generalizable to uninsured people or people with public insurance; potential errors or inconsistencies in diagnosis codes used to identify CF; information on inpatient admissions possibly being incomplete; and important assumptions that were made such as not accounting for off-label use of ivacaftor and inability to classify results by mutation class.

What's next? (50 words maximum)

As the US Food and Drug Administration expands use of data sources based on safety and effectiveness standards, people responsible for drug costs must make decisions around effectiveness and cost. It is important to conduct analyses as new medicines are developed so patients and providers have information regarding new therapies.