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Cystic Fibrosis Research News

Title:
Pancreatic enzyme prescription following ivacaftor licensing: a retrospective analysis of the US and UK cystic fibrosis registries

Lay Title:
Nationwide records suggest some people with CF have improved digestion with ivacaftor

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What was your research question?
Cystic fibrosis (CF) is a genetic disease affecting many organs, including the lungs and gut. In the past decade a new generation of treatments (modulators) was approved that improve lung health. We wanted to know whether they could also improve digestion, in particular, the role of the pancreas in digestion.

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Why is this important?

In 2022, people with CF and their care teams ranked improving gut problems in the top 3 most important issues to solve.

The pancreas normally makes enzymes to help digest food, but people with CF have a genetic change that stops the pancreas working normally. Most people need to take enzyme capsules to help them digest and absorb nutrients from food, but people can still have painful digestive symptoms.

In 2012, ivacaftor was approved to treat some people with CF, following trials which showed improvement in lung disease. However, we do not know if ivacaftor can also improve gut problems.

What did you do?

The use of ivacaftor and enzyme capsules are recorded on CF patient registries, which are vital nationwide records.

In this study, we looked at the large numbers of records from both the United States (US) and United Kingdom (UK) to see if ivacaftor led to a difference in the records of enzyme capsule use.

We compared use of enzyme capsules in those who were eligible to take ivacaftor versus those who were not eligible, before and after ivacaftor approval in 2012.

We also looked at enzyme capsule use specifically in people who did or did not receive ivacaftor.

What did you find?

In US records of people who were eligible for ivacaftor, we found the number of people taking enzyme capsules began to reduce after ivacaftor was approved, which was not seen in those ineligible for ivacaftor. When we looked in the US records at people who were specifically treated with ivacaftor, the chance of them taking enzyme capsules began to go down compared to people not on ivacaftor.

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Although we saw similar trends in UK records, the changes in the use of enzyme capsules were not reliably different when compared to people ineligible for ivacaftor or those not treated with it.

What does this mean and reasons for caution?

Findings from the US suggested that the number of people taking enzyme capsules went down after approval of ivacaftor. By 2017, 1 in 20 people taking ivacaftor had stopped taking enzyme capsules. People may have stopped receiving enzyme capsules after ivacaftor because their digestion had improved.

The changes seen in the US were not as strong when looking in UK records. Practices can vary between these countries, and there are big differences in the size of the CF populations. Our findings are also based on a record of capsule prescription, but not on whether the amount of capsules had changed.

What's next?

Ivacaftor was the first of the new generation of modulator treatments. Since then, combinations of modulators are available for many people with CF. The next steps are to test whether combinations lead to stronger evidence of improved digestion, and to understand more about the reasons for gut problems in CF.

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