European Cystic Fibrosis Society

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

Cystic Fibrosis Research News

Title:

RIOCIGUAT FOR THE TREATMENT OF PHE508DEL HOMOZYGOUS ADULTS WITH CYSTIC FIBROSIS

Lay Title:

A study of the drug riociguat as a possible treatment for people with cystic fibrosis

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What was your research question?

We wanted to see whether the drug riociguat might improve CFTR function and respiratory symptoms in people with cystic fibrosis (CF) and the F508del mutation.

Why is this important?

People with cystic fibrosis have defects in a gene called *CFTR*, which helps the body produce mucus, sweat, and other fluids. These defects mean that the protein made by the gene—also called CFTR—does not work properly, especially when both copies of the gene are affected. At the time the study took place, there were only a few treatments available to help the CFTR protein to work and they did not improve the situation very much. Therefore, we looked for a new treatment. Tests in animals show that riociguat might help CFTR to work properly.

What did you do?

We carried out a study in 31 adults with CF. People in the study were randomly chosen to take either riociguat or an identical tablet with no drug (a placebo) for 28 days. Neither the patient nor their doctors knew whether they were taking riociguat or the placebo. During the study we measured the amount of chloride in the patients' sweat, which shows how well CFTR is working. We also measured how well the patients' lungs were working. Patients were monitored throughout the study in case there were any important side effects.

What did you find?

After 28 days, sweat chloride levels increased slightly, regardless of whether patients were taking riociguat or placebo. There was no significant difference between the two treatments in measures to look for improvement (e.g. lung function). Most side effects were mild or moderate in severity. The types of side effects were as expected in people with CF and people taking riociguat.

What does this mean and reasons for caution?

In this study, there was no sign that riociguat might be effective for people with CF. Originally, a second part of the study was planned, using a higher dose of riociguat. However, as the data did not show even a slight sign of any improvement, it was thought unlikely that a higher dose would show an effect that would make a difference for patients. It is important to remember that this was a very small study and only lasted for 28 days. Therefore, it is not possible to be sure that riociguat cannot help some patients with CF.

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What's next?

Some new treatments have already become available for this group of patients with CF. Future clinical trials will continue to test new treatments for people with CF.

Original manuscript citation in PubMed

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