

Cystic Fibrosis Research News

Title:

Eluforsen: a potential new treatment option for people with cystic fibrosis (CF) due to the *F508del* gene mutation?

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What was your research question?

In this study we investigated whether a new drug called eluforsen may improve the function of the CFTR protein (Cystic Fibrosis Transmembrane Conductance Regulator) in patients with CF due to a mutation in the *F508del* gene.

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Why is this important?

This is important because in patients with CF, the CFTR protein is abnormal and does not work correctly. Eluforsen might lead to a better functioning CFTR protein and, as a result, a decrease in the symptoms of CF.

What did you do?

In this study, eluforsen was given to patients diagnosed with CF during a period of 4 weeks. The drug was taken 3 times per week via an intranasal device. Seven (7) patients in the study had 2 copies (homozygous group) and another 7 patients had 1 copy (heterozygous group) of the *F508del* gene mutation. We assessed the effect of eluforsen on the CFTR protein in both groups of patients by measuring chloride and sodium transport across the nasal mucosa (moist tissue lining the interior of the nose) using the Nasal Potential Difference (NPD) test. The NPD test result represents CFTR function and is sometimes used in the clinic to diagnose CF. It is often used to assess the potential efficacy or usefulness of new treatments for CF.

What did you find?

The NPD results indicated that eluforsen improved CFTR function in people with CF who had 2 copies of the *F508del* gene mutation (homozygous group). Significantly improved CFTR function was seen weeks 2 and 4 of treatment, and remained improved 2 weeks after stopping treatment. These results were supported by improved sodium transport, which was also observed after 2 weeks of treatment. Unfortunately, eluforsen did not improve CFTR function in people with CF with 1 copy of the *F508del* gene mutation (heterozygous group). No safety issues were described.

What does this mean and reasons for caution?

These encouraging results show that eluforsen may be a potential new treatment option for patients with CF carrying 2 copies of the *F508del* gene. However, the results we describe here are based on a small group of patients and further studies are necessary to learn more about the effect of eluforsen on CF symptoms.

What's next?

The results of this research support further studies using eluforsen to assess the effect of this potential treatment in a larger group of patients with CF with 2 copies of the mutated *F508del* gene.



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