

Cystic Fibrosis Research News

Title:

ANTISENSE OLIGONUCLEOTIDE ELUFORSEN IS SAFE AND IMPROVES RESPIRATORY SYMPTOMS IN F508DEL CYSTIC FIBROSIS

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What was your research question?

We investigated whether a new drug called eluforsen is safe and can be tolerated by people with cystic fibrosis (CF) with two copies of the F508del-*CFTR* mutation. We also looked at the effect of eluforsen on respiratory symptoms.

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Why is this important?

Studies measuring chloride and sodium transport in the surfaces of the nasal passages in people with CF and two copies of the *F508del*-mutation have shown that Eluforsen makes CFTR protein work better,. It is important to check if the drug is well-tolerated and safe when inhaled.

What did you do?

Eluforsen was given to adults with CF firstly as single doses and then in multiple doses (12 doses over four weeks). Four different doses of eluforsen were tested from a low-dose to a high-dose.

In both single dose and multiple dose studies safety was the main question but we also measured quality-of-life using the cystic fibrosis quality-of-life questionnaire respiratory symptom score (CFQ-R RSS) and performed lung function measurement.

What did you find?

Eluforsen was very well-tolerated and there was no concerns for safety at any dose that we tested. For the first three doses there was an increase in CFQ-R RSS of more than four points, which is considered to be the difference that is likely to be clinically meaningful; but as the highest dose there was no change in CFQ-R RSS. We did not see any meaningful changes in lung function.

What does this mean and reasons for caution?

The study confirmed that eluforsen is safe to use and suggests that there may be a clinical benefit from treatment. Although the study is too small to know this for sure, the results are encouraging.

What's next?

The results support further investigation of eluforsen to see the effect of inhaled eluforsen on cystic fibrosis symptoms in a larger group of people with two copies of the *F508del*-mutations.

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