

Cystic Fibrosis Research News

Title:

CFTR POTENTIATOR THERAPY AMELIORATES IMPAIRED INSULIN SECRETION IN CF PATIENTS WITH A GATING MUTATION

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What was your research question?

The question was whether drugs that improve the function of the chloride channel (potentiators) may improve the diabetic and metabolic status of patients with cystic fibrosis having the subset of mutations characterized by their decreased flow of chloride through the mutated channels also termed – "gating mutations".

Why is this important?

In recent years several new medications have been developed to improve ("potentiate") the function of the chloride channel that is mutated in cystic fibrosis and to improve lung disease in people with this condition. Last year it was shown that the function of the chloride channel (CFTR) in the pancreas is important for the secretion of insulin. We have now shown that Ivacaftor (a potentiator) that improved lung function as expected, also improved the diabetic profile of two people with cystic fibrosis who had gating mutations. It is very important to improve a person's diabetic profile since it has been shown that this has a large positive effect on the general health of people with CF.

What did you do?

We treated two patients who had gating mutations with ivacaftor for 16 weeks and compared their response to sugars (oral glucose tolerance test) from before treatment to the same test following treatment.



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What did you find?

Glucose (sugar) metabolism improved in both patients which seemed to be due to better insulin secretion during the first phase of typical insulin secretion – the time immediately following the increase in blood glucose in the blood.

What does this mean and reasons for caution?

This means that a treatment which improves the function of the chloride channel may also improve the function of the beta cell in the pancreas of people with cystic fibrosis-related diabetes and possibly also in other types of diabetes where insulin secretion not normal.

What's next?

A larger number of people are needed to take part in a study testing the metabolic response to glucose (sugar) of people with different cystic fibrosis mutations when they are treated with drugs developed to correct specific mutations.

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