

Cystic Fibrosis Research News

Title:

MyCyFapp clinical trial sub-study on change in PERT and fat absorption

Authors:

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What was your research question?

Is a new method to adjust the dose of enzyme supplements according to food characteristics effective in improving fat absorption in children with cystic fibrosis (CF)?

Why is this important?

Because most patients with CF suffer from pancreatic insufficiency, fat is not well digested and absorbed. Therefore, abdominal pains, fatty stools and poor weight gain are frequently observed. Using pancreatic enzymes is a very efficient treatment to address fat absorption,

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but there was no good way to optimize the dose, because the function varies according to the composition of meal.

What did you do?

First, fat digestion was studied in the lab to see how much dose of enzymes was needed for different food types. These data were integrated into a mathematical model that predicts the optimal dose of enzymes relative to food content, and incorporated this mathematical model into a mobile app that can be used by people with CF. We evaluated this method in 58 children with CF. We asked them to follow a 24h diet of their choice along with their regular doses of enzymes and register this information in the app. After one month of using the app, they were asked to repeat the same diet, but this time taking the dose of enzymes recommended by the mobile app. At both time points, faeces were collected, so we could measure the amount of absorbed fat.

What did you find?

We found that at the beginning of the study, the mean fat absorption was as high as 95% of the fat in the diet. Similarly, high values were obtained at the second time point. A relevant finding was a large variation in the doses of enzymes taken in the different countries at the beginning of the study. After one month of using the app, however, the doses changed and approached better to the recommendations under CF.

In addition, there were 12 children with lower fat absorption at the beginning of the study, with values below 85%. After one month of using the app and following the recommended doses, their coefficient of fat absorption significantly improved up to normal values around 94%.

What does this mean and reasons for caution?

We observed an overall decrease and less variability in the use of enzymes, which was able to maintain high fat absorption values in patients in a good fat absorption status, and could improve it in children with the poorest values. We did not identify any adverse effect. Therefore, we consider that the new method incorporated in the app can be safely used to adjust the dose of enzymes and maintain good values of fat absorption. Caution should be applied in children with specific gastrointestinal conditions not assessed in this study.



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What's next?

The next step is bringing the app into the clinical practice and make it available to all children with CF. This research team is actively searching for funding and administration procedures to make this implementation a reality.

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