

Cystic Fibrosis Research News

Title:

INHALED DRY POWDER MANNITOL IN CHILDREN WITH CYSTIC FIBROSIS: A RANDOMISED EFFICACY AND SAFETY TRIAL

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What was your research question?

Inhalation of mannitol twice daily via a dry powder inhaler makes the sticky cystic fibrosis (CF) airway secretions more liquid so that they can be cleared from the lungs more easily. In Europe mannitol treatment is approved for adults since mannitol leads to better lung function and less pulmonary exacerbations. We have now evaluated this treatment in children 6-18 years of age.

Why is this important?

In CF, lung disease starts at a young age. Therefore it is important that effective treatments are available for children.

What did you do?

92 children participated in this study. Half of the children first took mannitol twice daily for 8 weeks, and in the second treatment period they took an inactive drug (doctors call this 'a placebo'). The other half had the reverse order: first placebo, then mannitol.



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What did you find?

During mannitol treatment lung function improved: FEV1 was 3.42% higher compared to placebo and a measurement reflecting the smaller airways also improved (+4.97% in FEF25-75). During mannitol treatment, patients could expectorate more sputum and had less pulmonary exacerbations. Mannitol had this positive effect in children of all ages, even in children who were already inhaling Pulmozyme.

What does this mean and reasons for caution?

We now have proven that mannitol is an effective treatment in children with CF. Before starting treatment with mannitol, children must take a test dose as some get chest tightness when they inhale medication. This also happens with other inhaled drugs. In this study 86% of the patients could start the treatment.

What's next?

Hopefully mannitol treatment will soon become available for children. The European Medicines Agency, will decide whether mannitol can come on the market for children. Individual countries then decide on reimbursement of the treatment.

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