European Cystic Fibrosis Society

Journal of Cystic Fibrosis
The Official Journal of the European Cystic Fibrosis Society

Cystic Fibrosis Research News

Title:

TIOTROPIUM RESPIMAT® IN CYSTIC FIBROSIS: PHASE 3 AND POOLED PHASE 2/3 RANDOMIZED TRIALS

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What was your research question?

Tiotropium is an inhaled long-acting bronchodilator (a drug used to widen the airways for up to 24 hours) used in people with chronic obstructive pulmonary disease (COPD). In a previous trial, tiotropium improved lung function in people with cystic fibrosis (CF). We aimed to confirm the effectiveness and safety of tiotropium for treating CF in a second trial.

Why is this important?

People with CF suffer from blockages in the breathing tubes; bronchodilators relax the muscles in the lungs, widen the airways, and reduce the feeling of shortness of breath. Although bronchodilators are not approved for treating CF, they are widely used. Several studies have shown some bronchodilators work in people with CF, but not enough is known to recommend them for long-term treatment.

Tiotropium is one of the most commonly prescribed treatments for COPD and is also approved for treating asthma. It has the potential to open the airways and decrease breathing symptoms in people with CF.

What did you do?

This trial included people with CF of all ages (5 months–70.5 years) and disease severities. Children younger than 5 years were only included in the assessment of safety. Participants continued their usual CF treatment during the trial.

People with CF received either tiotropium (308 volunteers) or placebo (a dummy treatment containing no active medicine) (155 volunteers) via a Respimat inhaler for 12 weeks. Long-term safety was assessed over 12–60 weeks in an

extension of the trial where all the participants were treated with tiotropium and both they and the investigators knew this was the case.

Lung function, quality of life, occurrence of flare-ups of the disease (CF exacerbations) and safety and tolerability of tiotropium versus placebo were assessed for the current trial. The results were also combined with those from the previous trial.

What did you find?

Results from this trial showed trends for an improvement in lung function in volunteers receiving tiotropium. When combining results from both trials, lung function improved with tiotropium, in particular in volunteers aged 12 years and over. Volunteers had similar rates of exacerbations and a similar change in quality of life regardless of whether they received tiotropium or placebo. There were no relevant differences between the safety and tolerability of tiotropium and that of the placebo.

What does this mean and reasons for caution?

The effectiveness of tiotropium in people with CF, on top of usual CF treatment, was not clearly shown in this trial. When results from this and the previous study were combined, the improvements in lung function with tiotropium were not accompanied by beneficial effects on the occurrence of exacerbations or quality of life.

These studies, however, demonstrate that people with CF, including children of 5 years and younger, tolerated tiotropium well, and that the overall safety was consistent with that of tiotropium in people with COPD.

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What's next?

The results suggest that treating some people with CF, especially those aged 12 years and older and those with reduced lung function, with tiotropium can achieve better outcomes. Further studies are needed to confirm this.

Original manuscript citation in PubMed

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