

Cystic Fibrosis Research News

Title:

A TREATMENT EVALUATOR TOOL TO MONITOR THE REAL-WORLD EFFECTIVENESS OF INHALED AZTREONAM LYSINE IN CYSTIC FIBROSIS

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What was your research question?

How do people with cystic fibrosis (CF) who are seen in real-world clinics respond when they start a particular inhaled antibiotic (aztreonam lysine for inhalation)? Aztreonam lysine for inhalation is used for the chronic suppression of lung infections caused by the bacteria *Pseudomonas aeruginosa*, which is common in people with CF. Our aim was to develop an easy-to-use, computer-based tool to record this information.

Why is this important?

Clinical trials test whether a therapy works and is well-tolerated by patients under strict conditions, which do not necessarily reflect the experience of patients in real-life. Our tool collected data about real-world patients who were prescribed aztreonam lysine in nine CF centres in the UK and Ireland.

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What did you do?

Respiratory doctors from nine large cystic fibrosis centres in the UK and Ireland met to discuss and agree on the information that should be recorded by the tool. Following this, we developed an online, user-friendly interface for use by the nine clinics to collect patient data, which was recorded whenever patients attended a routine clinic appointment. We then analysed data from 12 months before and 12 months after the patients started aztreonam lysine to determine how they responded to this therapy.

What did you find?

Compared with the previous 12 months, starting aztreonam lysine meant that patients' lung function improved and they gained weight. They also had a significantly fewer flare-ups of lung disease (exacerbations), which were measured by looking at the time patients spent in hospital and on antibiotics by infusion.

What does this mean and reasons for caution?

An important goal of CF treatment is to reduce the number of exacerbations a patient experiences, which can lead to frequent periods when they are in hospital for weeks at a time, interfering with work and home life and potentially causing a decrease in lung function that does not recover afterwards. The increases in lung function and body weight which were seen are also important as they are linked to healthier patient lives. It is important to bear in mind that, as this was an observational study of routine clinical practice, there was no comparator group who were not prescribed aztreonam lysine. Therefore conclusions cannot be drawn about the cause of these changes, only that there was a benefit after the treatment started. Additionally, we did not measure whether patients took the aztreonam lysine as directed once it was prescribed.

What's next?

Our tool has the potential to be used to collect data on other CF therapies and to be adapted to collect a range of clinical, economic and patient-related information. For example, it could be used to analyse and compare different combinations of inhaled antibiotics in real-life patients, which could help doctors to prescribe the most appropriate therapies for patients.



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