Azithromycin for long-term treatment of cystic fibrosis

Information for the public
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About this information

This information explains the evidence summary about the off-label use of azithromycin for the long-term treatment of cystic fibrosis. The evidence summary is an overview of the available information about this medicine. It aims to help prescribers and patients when they are considering whether or not to use an unlicensed or off-label treatment. The summary does not contain recommendations from NICE on whether the medicine should be used.

Licensing medicines

In the UK, medicines need to have a licence before they can be marketed. To get a licence, the manufacturer of the medicine has to provide evidence that shows that the medicine works well enough and is safe enough to be used for a specific condition and for a specific group of patients, and that they can manufacture the medicine to the required quality. Medicines can be prescribed without a licence (an 'unlicensed medicine') if there is no suitable licensed alternative and it is likely to benefit the patient.

A medicine can also be prescribed 'off-label'. This means the prescriber wants to use it in a different way than is set out in the terms of its licence. This could mean using the medicine for a different condition or a different group of patients, or it could mean a change in the dose or that the medicine is taken in a different way. There is more information about licensing medicines on NHS Choices.
What is cystic fibrosis?

Cystic fibrosis is an inherited disease that affects the lungs by clogging them with thick and sticky mucus. This affects breathing and increases the risk of lung infections from bacteria (most commonly *Pseudomonas aeruginosa*). Persistent lung infection is known to reduce the length and quality of life of people with cystic fibrosis. Therefore, treatments such as physiotherapy and antibiotics are used to prevent and control lung infections, reduce the number of times people are admitted to hospital, and improve quality of life.

About azithromycin

Azithromycin is an antibiotic. In the UK, it is licensed for treating certain infections (bronchitis, pneumonia, sinusitis, pharyngitis and tonsillitis, otitis media, skin and soft tissue infections and genital infections). It is usually only taken for 3 days. When azithromycin is used for treating cystic fibrosis, this use is known as off-label.

Some guidelines that doctors use when treating cystic fibrosis advise that they should consider a 6-month trial of azithromycin in people with cystic fibrosis who are deteriorating on usual treatments.

It isn't clear how azithromycin works in cystic fibrosis. It may reduce inflammation in the lungs and prevent *Pseudomonas aeruginosa* bacteria sticking to cells in the lungs.

Summary of possible benefits and harms

*How well does azithromycin work?*

A review of 10 different studies, which all looked at how well azithromycin worked for treating cystic fibrosis, has been published. These studies included 959 children and adults between them, and they all looked at azithromycin in different ways.

In 4 of the studies, people who were taking azithromycin for 6 months found that their lungs improved more than in those who were taking dummy tablets (these don't have any medicine or antibiotic in them and are also known as a placebo). The number of chest infections in people taking azithromycin for 6 months was half of that in the people taking dummy tablets. However, these infections were recorded in different ways in the studies meaning the results were difficult to compare.
In 2 of the studies, the same numbers of people were admitted to hospital for treatment for their cystic fibrosis regardless of whether they were taking azithromycin or the dummy tablets.

Some of the studies also looked at whether taking azithromycin reduced the need to take other antibiotics to treat an infection. In 3 studies, taking azithromycin instead of the dummy tablets reduced the need for additional antibiotics that can be taken by mouth (for example, tablets) to treat infections. However, taking azithromycin didn't make any difference for people who needed antibiotics by an injection to treat infections.

It wasn't clear whether treating cystic fibrosis with azithromycin affected a person's quality of life. Also, there was little evidence to show how well azithromycin works when it is taken for more than 6 months. It isn't known how well azithromycin works in children who are under 6 because no children of this age group were included in the studies.

**What are the possible harms or side effects?**

There is little information about how safe it is to take azithromycin over a longer period of time for treating cystic fibrosis. However, some of the studies that were looked at lasted 12 months and no serious side effects were reported.

Diarrhoea, stomach pain, nausea (feeling sick) and wind (flatulence) are very common with azithromycin (occurring in at least 1 in 10 people).

Sometimes people have hearing loss after taking azithromycin for a long time, although hearing does return after stopping treatment. Occasionally, azithromycin can cause abnormal heart beats in some people.

Please note that the results of the research studies only indicate the benefits and harms for the population in the studies. It is not possible to predict what the benefits and harms will be for an individual patient being treated with azithromycin.

**Prescribing azithromycin**

If a prescriber wants to use an unlicensed or off-label medicine, they must follow their professional guide, for example for doctors the General Medical Council's [good practice guidelines](#). These include giving information about the treatment and discussing the possible benefits and harms so that the patient has enough information to decide whether or not to have the treatment. This is called giving informed consent.
A full version of the summary aimed at healthcare professionals is available on the NICE website. The summary for healthcare professionals does not contain recommendations from NICE on whether the medicine should be used.

Questions to ask

- Why am I being offered an off-label medicine?
- What does the treatment involve?
- What are the benefits I might get?
- How good are my chances of getting those benefits?
- Could having the treatment make me feel worse?
- Are there alternative treatments?
- What are the risks of the treatment?
- Are the risks minor or serious? How likely are they to happen?
- What may happen if I don't have the treatment?

More information

NICE has published information about how evidence summaries for unlicensed and off-label medicines are developed.

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