EMA/450625/2012
EMEA/H/C/000996

EPAR summary for the public

Cayston

aztreonam

This document is a summary of the European public assessment report (EPAR) for Cayston. It explains how the Committee for Medicinal Products for Human Use (CHMP) assessed the medicine to reach its opinion in favour of granting a marketing authorisation and its recommendations on the conditions of use for Cayston.

What is Cayston?

Cayston is a powder and solvent that are made up into a nebuliser solution. It contains the active substance aztreonam.

What is Cayston used for?

Cayston is used to suppress long-term lung infection caused by *P. aeruginosa* bacteria in adults and children aged six years or older with cystic fibrosis.

Cystic fibrosis is an inherited disease that affects the cells that secret mucus in the lungs and the cells that secret digestive juices from the glands in the gut and pancreas. In cystic fibrosis these secretions become thick, blocking the airways and the flow of digestive juices. This leads to problems with the digestion and absorption of food, resulting in poor growth, and long-term infection and inflammation of the lungs because of excess mucus not being cleared away.

Because the number of patients with bacterial lung infection in cystic fibrosis is low, the disease is considered 'rare', and Cayston was designated an 'orphan medicine' (a medicine used in rare diseases) on 21 June 2004.

The medicine can only be obtained with a prescription.

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How is Cayston used?

Cayston is to be used with a nebuliser (a special machine that changes the solution into an aerosol that the patient can breathe in). Cayston is given three times a day for four weeks, with at least four hours between each dose. A bronchodilator (a medicine that widens the airways in the lungs) should be used before each dose of Cayston. If the patient takes multiple inhaled treatments the bronchodilator should always be taken first, followed by a mucolytic (a medicine to make mucus thinner) and Cayston last. If the doctor thinks that further treatment cycles with Cayston are needed after the first course, a gap of four weeks is recommended after every four-week treatment cycle with Cayston.

How does Cayston work?

In cystic fibrosis, the patient's lungs produce too much thick mucus, which allows bacteria to grow more easily. In patients with cystic fibrosis, *P. aeruginosa* infections usually start in the first 10 years of life and can cause long-term lung problems.

The active substance in Cayston, aztreonam, is an antibiotic that belongs to the group 'beta-lactams'. It works by attaching to proteins on the surface of the *P. aeruginosa* bacteria. This prevents the bacteria from building their cell walls, which kills them.

Aztreonam has been available as an injection since the 1980s as an 'arginine salt'. In Cayston, aztreonam is available as a 'lysine salt', which makes it possible for the antibiotic to be breathed directly into the lungs without causing irritation.

How has Cayston been studied?

Cayston was compared with placebo (a dummy treatment) in two main studies involving a total of 375 patients with cystic fibrosis who had long-term lung infection due to *P.aeruginosa*, most of whom were adults. In the first study, the main measure of effectiveness was how long it took before the patients needed other antibiotics by inhalation or injection into a vein. In the second study, the main measure of effectiveness was how the patients rated their respiratory symptoms on a standard scale for cystic fibrosis. In these two studies, patients were treated for four weeks.

A third main study involving 268 patients (including 59 children from six to 17 years of age) compared Cayston with another inhaled antibiotic (tobramycin nebuliser solution). The main measure of effectiveness was based on improvements in patients' forced expiratory volumes (FEV_1, the maximum volume of air a person can breathe out in one second).

An additional study compared four weeks of Cayston with placebo in patients with mild lung disease due to cystic fibrosis. It looked at how well the lungs worked, the amount of *P. aeruginosa* bacteria in the patients' mucus and their respiratory symptoms.

What benefit has Cayston shown during the studies?

Cayston was more effective than placebo at suppressing lung infection caused by *P. aeruginosa* bacteria in patients with cystic fibrosis. In the first study, the patients who received Cayston required other antibiotics after 92 days, compared with 71 days for the patients who received placebo. In the second study, respiratory symptoms improved in the patients who received Cayston, compared with the patients who received placebo.

The third study showed that Cayston compared favourably with the comparator antibiotic: after four weeks of treatment, the increase in FEV_1 adjusted for age, height and sex was 8.35% with Cayston and 0.55% with the comparator; after three

treatment cycles, the increase with Cayston was 2.05% compared with a decrease of 0.66% with the comparator. An improvement in lung function was also seen in children aged six to 17 years, both after four weeks of treatment and after three treatment cycles.

The additional study produced results that were consistent with those of the main studies.

What is the risk associated with Cayston?

The most common side effects with Cayston (seen in more than 1 patient in 10) are wheezing, cough, pharyngolaryngeal pain (pain in the throat and voice box), nasal congestion (blocked nose), dyspnoea (difficulty breathing) and fever. For the full list of all side effects reported with Cayston, see the package leaflet.

Cayston must not be used in people who are hypersensitive (allergic) to aztreonam or any of the other ingredients.

Why has Cayston been approved?

The CHMP concluded that there was an unmet medical need for new antibiotics in patients with cystic fibrosis, since many of these patients have already developed resistance to other antibiotics by the time they reach adulthood, and because *P. aeruginosa* lung infections are a cause of severe health problems in patients with cystic fibrosis. The CHMP decided that Cayston's benefits are greater than its risks and recommended that it be given marketing authorisation.

Other information about Cayston:

The European Commission granted a marketing authorisation valid throughout the European Union for Cayston on 21 September 2009.

The full EPAR for Cayston can be found on the Agency's website: ema.europa.eu/Find medicine/Human medicines/European public assessment reports. For more information about treatment with Cayston, read the package leaflet (also part of the EPAR) or contact your doctor or pharmacist.

The summary of the opinion of the Committee for Orphan Medicinal Products for Cayston can be found on the Agency's website: ema.europa.eu/Find medicine/Human medicines/Rare disease designation.

This summary was last updated in 07-2012.