Single Technology Appraisal (STA)

Mannitol dry powder for inhalation for the treatment of cystic fibrosis [ID85]

Cystic Fibrosis Trust response

The Cystic Fibrosis Trust is the UK national charity for people living with Cystic Fibrosis. The Trust funds world-leading medical research, ensures safe and appropriate clinical care, and offers direct support for people with Cystic Fibrosis and their families.

Cystic Fibrosis is an inherited and progressive life-limiting disease which affects internal organs (particularly the lungs and digestive system) by clogging them with thick sticky mucus. This makes it harder to breathe and to digest food. The mucus in the lungs provides an ideal environment for pathogenic bacteria, promoting recurrent and increasingly frequent respiratory infections. In 2009, the average age at death was only 27.

Response to the consultation

The Cystic Fibrosis Trust is very disappointed by NICE's initial decision not to recommend mannitol dry powder for inhalation for the treatment of cystic fibrosis.

We firmly believe that not enough emphasis has been placed on how mannitol will help to relieve the burden of treatment and care for people with Cystic Fibrosis, and therefore this important evidence has not been taken into account when arriving at the initial decision.

People with Cystic Fibrosis routinely have to undergo hours of treatment and physiotherapy every day. Maintaining this regime is time consuming, exhausting and impacts on quality of life. New treatments that help to alleviate this burden, encourage adherence and therefore improve clinical outcomes, have been slow to come through the therapeutic pipeline. Cystic Fibrosis clinicians need more treatment options to be made available for people with CF. mannitol would be available to a broad population of CF patients, irrespective of microbiological status. This is the first innovation to be approved to tackle the fundamental issue of airway clearance in 18 years.

This treatment is a step change in terms of improving quality of life for people with CF, specifically because mannitol is a dry powder inhaled treatment that is very quick and easy to administer. The importance of this new application cannot be overstated in terms of convenience and ease of use. The drug delivery device can be carried in a small bag and administered at times convenient to the person with CF. The drug is also easy to take at the right dose and frequency, important factors in improving adherence, so that the full benefit of the treatment can be received.

Currently, mannitol would offer the only alternative to nebulised hypertonic saline which is significantly more time consuming and less convenient when taking into

account the need for preparation and repeated cleaning. Also, it is not well tolerated in all patients, increasing cough and wheeze significantly and has an unpleasant taste when inhaled via nebuliser. The CF Trust regularly hears from people with CF that these factors negatively impact on adherence and are likely, therefore, to lead to poorer clinical outcomes.

This treatment is also a step change in terms of its effectiveness in CF. It has a clear clinical benefit as it limits lung damage by reducing exacerbations by 24 per cent. Exacerbations are now accepted as a major factor in long term FEV1 decline. The goal for most adult CF patients is to maintain FEV1 for as long as possible and any improvement in an already heavily treated patient group is dramatic. Therefore, FEV1 improvements of greater than 100ml are meaningful in CF. The importance of maintaining and improving FEV1 cannot be underestimated both in terms of improving health outcomes and improving quality of life. Poor lung function and frequent exacerbations increase the likelihood of needing intravenous antibiotics and time spent in hospital, which not only has a significant impact on people with CF, but also their family and carers. Reduced time spent in hospital also help people with CF stay away from the risks of cross infection, which is also key to improved long term outcomes and survival. It also means that they are able to continue to work and live their lives as normally as possible.

The treatment burden in CF is such that any treatments that are not being adhered to or that are not having a clinical benefit will stop being prescribed. Treatments that don't produce a benefit would routinely be stopped by CF clinicians. As stated above, people with CF have a huge burden of treatment and physiotherapy to endure everyday. If they feel that a treatment is not working they will discuss this with their clinical team.

The proposed introduction of mannitol represents a small budget impact in the context of other treatments currently available for CF and would benefit a broad population of CF patients. In fact, there could be potential cost savings to be made from reducing the need for hospitalisation due to repeat exacerbations.

Therefore, the Cystic Fibrosis Trust is of the opinion that the provisional recommendations from NICE regarding mannitol are not a sound and a suitable basis for guidance to the NHS.