NATIONAL INSTITUTE FOR HEALTH AND CLINICAL EXCELLENCE

Multiple technology appraisal (MTA)

Colistimethate sodium powder and tobramycin powder for inhalation for the treatment of pseudomonas lung infection in cystic fibrosis [ID342]

Thank you for agreeing to give us your views on the technology and the way it should be used in the NHS.

Patients and patient advocates can provide a unique perspective on the technology, which is not typically available from the published literature.

To help you give your views, we have provided a template. The questions are there as prompts to guide you. You do not have to answer every question. Please do not exceed the 8-page limit.

About you

Your name: Lynsey Morton

Name of your organisation: Cystic Fibrosis Trust

Are you (tick all that apply):

X a patient with the condition for which NICE is considering this technology?

- a carer of a patient with the condition for which NICE is considering this technology?
- an employee of a patient organisation that represents patients with the condition for which NICE is considering the technology? If so, give your position in the organisation where appropriate (e.g. policy officer, trustee, member, etc)
- other? (please specify)

NATIONAL INSTITUTE FOR HEALTH AND CLINICAL EXCELLENCE

Multiple technology appraisal (MTA)

What do patients and/or carers consider to be the advantages and disadvantages of the technology for the condition?

1. **Advantages**

(a) Please list the specific aspect(s) of the condition that you expect the technology to help with. For each aspect you list please describe, if possible, what difference you expect the technology to make.

The treatment will be quicker and simpler to administer.

It will be portable and therefore taken with the patient everywhere with ease and fit into their everyday lifestyle. It may even be small enough to put in a handbag for instance.

An inhaler will be smaller and more discreet than a nebuliser, (this is particularly important with image conscious teens and young adults.)

Convenience. The inhaler won't need an electric supply. Unlike a nebuliser, it won't require components, servicing, charging, syringes/needles and vials.

It will help to increase independence. In terms of less preparation time, sterilisation etc some people with CF can do this themselves others rely on a carer. An inhaler can be self administered with ease. Often the cleaning prep of nebulisers takes longer than the treatment itself.

It will help with adherence as adults and children with CF already take many medications and treatments and therefore poor compliance is common. Anything that makes the treatment quicker and easier would be helpful to improve adherence and the burden of care.

.

Powder may be tolerated better than nebulised vapour.

(b) Please list any short-term and/or long-term benefits that patients expect to gain from using the technology. These might include the effect of the technology on:

- the course and/or outcome of the condition
- physical symptoms
- pain
- level of disability
- mental health

NATIONAL INSTITUTE FOR HEALTH AND CLINICAL EXCELLENCE

Multiple technology appraisal (MTA)

- quality of life (lifestyle, work, social functioning etc.)
- other quality of life issues not listed above
- other people (for example family, friends, employers)
- other issues not listed above.

Long term:

Improved health; taking the treatment is preventative to help slow down any further deterioration caused by infection on the lung and lung damage. This could potentially help to increase overall life span, but also the quality of life allowing the person with CF to continue with their everyday activities.

For those who are colonised it may help reduce cough and help prevent lung infections. This may therefore reduce the number of exacerbations and may mean less time in hospital.

Better quality of life and life expectancy outlook.

It also may be beneficial long term for compliance as a patient may be more likely to regularly take the treatment if its simple to assemble (or no assembly or preparation time is required), quick and easy to use and is small and discreet.

CF is a long term condition. We already take many treatments on a daily basis. Anything that can help reduce burden and improve ease and time is certainly welcomed.

Short term:

It will be small, portable and easy to use at work or school for those patients who need something to fit in with their busy lifestyles and take it alongside the many other treatments for their CF.

Reduced burden of cleaning, sterilising, use of electric, storage of bulky equipment

Quicker and simpler to do alongside other CF related treatments so that burden of treatment and time spent preparing/administering is reduced.

Eradication of pseudomonas (for those not colonised).

NATIONAL INSTITUTE FOR HEALTH AND CLINICAL EXCELLENCE

Multiple technology appraisal (MTA)

What do patients and/or carers consider to be the advantages and disadvantages of the technology for the condition? (continued)

2. **Disadvantages**
Please list any problems with or concerns you have about the technology.
Disadvantages might include:

- aspects of the condition that the technology cannot help with or might make worse.
- difficulties in taking or using the technology
- side effects (please describe which side effects patients might be willing to accept or tolerate and which would be difficult to accept or tolerate)
- impact on others (for example family, friends, employers)
- financial impact on the patient and/or their family (for example cost of travel needed to access the technology, or the cost of paying a carer).

People with CF who are not in full time education, on higher rate benefits or have CF related diabetes already have to pay for prescriptions via a yearly certificate. The difficulty would be if the treatment was high costs and not included alongside the other drugs, which would potentially create a postcode lottery with some patients having more access to the drug than others depending on where you live.

There may be side effects, however what may be acceptable to one patient may not be acceptable to another, depending on how active and mobile that person is and at what stage in their CF they are. Patients could potentially choose to revert back to alternative treatment technologies if they do not tolerate the inhaled format.

The technology is likely to be used by those who are colonised with pseudomonas or those who have been diagnosed for initial eradication. Therefore not all people with CF will be on this treatment and may still be on nebulisers for other treatments. Those CF patients who are colonised will remain on the treatment for the rest of their lives to help prevent further infection and deterioration of the lungs.

Very small children and babies may not be able to take the inhaler format.

NATIONAL INSTITUTE FOR HEALTH AND CLINICAL EXCELLENCE

Multiple technology appraisal (MTA)

3. Are there differences in opinion between patients about the usefulness or otherwise of this technology? If so, please describe them.

Generally inhalers are viewed to be quicker easier, portable and more convenient than a nebuliser or intravenous antibiotics.

CF is a life threatening condition and treatments need to be taken on a daily basis for the rest of a person's life, so the technology is moving forward to something that is going to help reduce the burden and the time spent doing treatments.

The treatment will help treat and prevent further deterioration from pseudomonas, which if left untreated will cause significant lung damage, resulting in more hospital treatment and intervention and reduce life expectancy further.

4. Are there any groups of patients who might benefit **more** from the technology than others? Are there any groups of patients who might benefit **less** from the technology than others?

Those who may benefit more are those who are non-compliant currently with their nebulisers because of factors such as lack of time, or who are on a substantial amount of treatments for CF and related complications.

There may be some cases where side effects of the new technology may mean that a patient may wish to choose chose an alternative method.

NATIONAL INSTITUTE FOR HEALTH AND CLINICAL EXCELLENCE

Multiple technology appraisal (MTA)

Comparing the technology with alternative available treatments or technologies

NICE is interested in your views on how the technology compares with existing treatments for this condition in the UK.

(i) Please list any current standard practice (alternatives if any) used in the UK.

Nebulisers (ie: i-neb, eflow, porta-neb and compressors)
Intravenous antibiotics via drip (at home or as an inpatient)

(ii) If you think that the new technology has any **advantages** for patients over other current standard practice, please describe them. Advantages might include:

- improvement in the condition overall
- improvement in certain aspects of the condition
- ease of use (for example tablets rather than injection)
- where the technology has to be used (for example at home rather than in hospital)
- side effects (please describe nature and number of problems, frequency, duration, severity etc.)

The technology mainly would help better compliance which could help more effective eradication and better management of condition.

In turn this could help reduce exacerbations and time spent in hospital.

This could help to increase life expectancy, independence and overall quality of life.

I am unsure of if the inhaled format is any more effective at reaching the lungs than the nebulised version?!

It is portable unlike the larger version which needs to be sterilised, charged and has separate compartments/vials etc.

NATIONAL INSTITUTE FOR HEALTH AND CLINICAL EXCELLENCE

Multiple technology appraisal (MTA)

(iii) If you think that the new technology has any **disadvantages** for patients compared with current standard practice, please describe them. Disadvantages might include:

- worsening of the condition overall
- worsening of specific aspects of the condition
- difficulty in use (for example injection rather than tablets)
- where the technology has to be used (for example in hospital rather than at home)
- side effects (for example nature or number of problems, how often, for how long, how severe).

Side effects. The potential for side effects or personal preference by the patient may be a disadvantage but this would be dependant on the patient as we all have different tolerance levels and they may wish to use an alternative method.

High cost?! The other potential disadvantage may arise if some patients are easily able to be prescribed it whilst others with CF are not due to high cost implications depending on who is prescribing it and where.

Research evidence on patient or carer views of the technology

If you are familiar with the evidence base for the technology, please comment on whether patients' experience of using the technology as part of their routine NHS care reflects that observed under clinical trial conditions.

Are there any adverse effects that were not apparent in the clinical trials but have come to light since, during routine NHS care?

NATIONAL INSTITUTE FOR HEALTH AND CLINICAL EXCELLENCE

Multiple technology appraisal (MTA)

Are you aware of any research carried out on patient or carer views of the condition or existing treatments that is relevant to an appraisal of this technology? If yes, please provide references to the relevant studies.

NATIONAL INSTITUTE FOR HEALTH AND CLINICAL EXCELLENCE

Multiple technology appraisal (MTA)

Availability of this technology to patients in the NHS

What key differences, if any, would it make to patients and/or carers if this technology was made available on the NHS?

Many of the reasons that I have already stated. The main reason being that it would increase adherence because is easier, simpler and faster to manage than a nebuliser. This could mean less time spent in hospital as an inpatient, intravenous treatments and overall deterioration.

It would mean a better quality of life by significantly helping to reduce the burden of care, particularly as

a. It is a life long illness
b. There are already many other medications and treatments to take
c. Many work/students or rely on carers so it would help independence
d. Is it lower maintenance and less/no preparation time

What implications would it have for patients and/or carers if the technology was **not** made available to patients on the NHS?

Many patients would continue to be non-compliant with current treatments. This could mean more hospital input and intravenous treatment is required.

Not taking treatments in the long term could also result in poorer lung function and ultimately a shorter life expectancy and quality of life.

Treatment would take longer and be more complicated to take

Treatment would be high maintenance i.e.: charging, sterilising, drawing up of medication etc.

Treatment is currently portable, but not as small and discreet as an inhaler and still requires a lot of accessories and equipment ie: charger, vials, syringes etc.

NATIONAL INSTITUTE FOR HEALTH AND CLINICAL EXCELLENCE

Multiple technology appraisal (MTA)

Are there groups of patients that have difficulties using the technology?

Those who are non complaint because they are overwhelmed with the number of different treatments that they need to take on a daily basis for the rest of their lives.

Unsure if inhaler would be suitable for very small children??!

Other Issues

Please include here any other issues you would like the Appraisal Committee to consider when appraising this technology.

The change from a nebulizer to an inhaler is so significant for the CF population. We are predominantly children and teenagers and young adults. Many have issues with compliance because of the sheer volume of medication that we have to take, prepare and administer on a daily basis.

The lungs are the main cause of death for people with CF, and therefore effective management of the lungs and keeping them infection free is very important.

ANYTHING that can help reduce this burden and make things easier and quicker to take is welcomed because it means that we are more likely to take it and this means we will ultimately spend less time in hospital and that deterioration will be delayed.

CF is a life long illness of slow deterioration and is with us for the rest of our lives, which means many of these treatments will be too, so it is important that if there are any technologies that help us to take our treatments that they are made available so that we can continue to live longer and have a better quality of life.