# NATIONAL INSTITUTE FOR HEALTH AND CLINICAL EXCELLENCE

## Single technology appraisal (STA)

## Mannitol dry powder for inhalation for the treatment of cystic fibrosis

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: Emma Lake**

**Name of your organisation:**

**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?

X 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)

Senior Clinical Care Patient Adviser

- other? (please specify)

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**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.

Patients and carers expect this technology to help with mucus clearance during physiotherapy treatment, improving lung function and reducing the risk if infection and long term damage to the lungs. Improved lung function means longer and better quality of life with less time spent in hospital, increased exercise tolerance and improved quality of life.

(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
- 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.

The effects on course and outcome of condition: please see answer to (a) above
Physical Symptoms: reduced cough and exacerbations. Physiotherapy treatment would become easier, due to the powder thinning the mucus.
Quality of life: As this is an inhaled powder this would be quick and easy to use when compared to current mucolytics which are nebulised. Quicker treatment times improve lifestyle.

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**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).

There may be difficulties in using the hand held device for those with physical disabilities.
Possible side affects of increased wheeze may not be tolerated in all patients.

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

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?

**Comparing the technology with alternative available treatments or technologies**

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

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

nebulised hypertonic saline
nebulized mucolytic rhDNase

(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.)

Over current standard practice Mannitol has the advantage for patients regarding ease of use. Device is smaller and does not require mains power or batteries to work. Device is cost free to patient, which is currently not the case for all nebuliser devices used for current treatments. Treatment time would be greatly reduced.

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(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).

**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?

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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.

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**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?

Improved outcomes of the condition and improved quality of life. Those with CF have a high burden of care and any new treatment which can replace current treatments while improving outcomes and shorten treatment times would be welcomed.

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

Continued cost of nebuliser device for some patients adding financial strain on patients and families. Continued decreased quality of life due to treatment burden meaning patients and families are unable to contribute fully to working, home and social life.

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

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**Other Issues**

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