NATIONAL INSTITUTE FOR HEALTH AND CARE EXCELLENCE

Centre for Health Technology Evaluation

# Highly Specialised Technologies Evaluation Committee (HSTEC) meeting minutes

**Minutes:** Confirmed

**Date:** Thursday 08 September 2022

**Location:** Via Zoom

## Attendees

Committee members present

1. Dr Peter Jackson (Chair) Present for all items
2. Professor Philip Beales Present for all items
3. Annett Blochberger Present for all items
4. Emtiyaz Chowdhury Present for all items
5. Stuart Davies Present for all items
6. Sarah Davis Present for all items
7. Jeremy Manuel O.B. E Present for all items
8. Dr Shehla Mohammed Present for all items
9. Anthony Williams Present for all items
10. Professor Ed Wilson Present for all items

NICE staff (key players) present

Jasdeep Hayre, Associate Director Present for all items

Celia Mayers, Project Manager Present for all items

Christian Griffiths, Heath Technology Assessment Adviser Present for all items

Alan Moore, Heath Technology Assessment Analyst Present for all items

Emeline Coventry, Senior Medical Editor Present for all items

Mandy Tonkinson, Public Involvement Adviser, PIP Items 1 to 4.1.3

Rosalee Mason, Coordinator, MIP Items 1 to 4.1.3

Gemma Smith, Coordinator, COT Present for all items

Leah Kelly, Administrator, TA Present for all items

NICE staff (observers) present

Catrin Austin, Technical Analyst, Methods, & Economics team Present for all items

Emily Eaton-Turner, Technical Adviser, Commercial Liaison team Present for all items

Neha Jiandani, Technical Analyst, Commercial Liaison team Present for all items

Philip Ranson, Communications Manager, Media Relations team Present for all items

Leo Koeser, Scientific Adviser, Scientific Advice team Present for all items

External assessment group representatives present

Paul Tappenden, School of Health and Related Research (ScHARR), Items 1 to 4.2.1

Abdullah Pandor, School of Health and Related Research (ScHARR), Items 1 to 4.2.1

Clinical, Patient & NHS England experts present

Dr Ayesha Ali, Medical Advisor in Highly Specialised Services, NHS England, Items 1 to 4.1.3

Rob Burley, Patient expert nominated by Muscular Dystrophy UK, Items 1 to 4.1.3

Mark Silverman, Patient expert nominated by Action Duchenne, Items 1 to 4.1.3

Katherine Wedell, Patient expert nominated by Action Duchenne, Items 1 to 4.1.3

Dr Anne-Marie Childs, Consultant Paediatric Neurologist, clinical expert nominated by PTC Therapeutics, Items 1 to 4.1.3

## Minutes

### Introduction to the meeting

The chair Dr Peter Jackson welcomed members of the committee and other attendees present to the meeting.

The chair noted apologies from Dr Paul Arundel, Carrie Gardner, and Dr Mark Sheehan

### News and announcements

None.

### Minutes from the last meeting

The committee approved the minutes of the committee meeting held on Wednesday 6 July 2022.

### Evaluation of ataluren for treating Duchenne muscular dystrophy with a nonsense mutation in the dystrophin gene - review of HST3 [ID1642]

* 1. Part 1 – Open session
     1. The chair welcomed the invited experts, external assessment group representatives, members of the public and company representatives from PTC Therapeutics.
     2. The chair asked all committee members and experts, external assessment group representatives and NICE staff present to declare any relevant interests in relation to the item being considered.
* Committee member Professor Ron Akehurst declared financial Interest as his company Lumanity is working with PTC Therapeutics and he has been involved in the work. As a direct conflict Professor Akehurst did not take part in this meeting.
* Committee member Emtiyaz Chowdhury declared non-financial professional & personal Interest as Parexel is a CRO which works with the manufacturer for HST3 and HST6. However, he is not aware of any work that Parexel have done for this specific technology, nor has he or his team been involved in any work related to this technology. It was agreed his declaration would not prevent Emtiyaz from participating in discussions on this appraisal
* Committee member Sarah Davis declared financial interests as she is a member of Scharr-tag the EAG on this appraisal, but she has had no involvement with their work.it was agreed her declaration would not prevent Sarah from participating in discussions on this appraisal.
* Nominated patient expert Mark Silverman a direct non-financial interest as he I am a trustee of Action Duchenne. It was agreed that his declaration would not prevent Mark from providing expert advice to the committee.
* Nominated clinical expert Dr Anne-Marie Childs declared Direct financial and non-financial interests as she has previously contributed to advisory boards for PTC Therapeutics and has been sponsored to attend educational meetings and given invited lectures on their behalf. Last year I was asked to present the current data from the STRIDE study to the national investigators meeting, in the last 5 yrs, she has also contributed to advisory boards for Roche, Biogen, and Novartis in relation to novel therapies in SMA as is a PI for a number of commercial studies in DMD, including trials sponsored by Sarepta and Fibrogen. It was agreed that her declaration would not prevent Dr Childs from providing expert advice to the committee.
* No further interests were declared for this evaluation.
  + 1. The Chair led a discussion of the evidence presented to the committee. This information was presented to the committee by Professor Ed Wilson, Dr Shehla Mohammed and Jeremy Manuel O.B. E.
  1. Part 2a – Closed session (company representatives, professional experts, and members of the public were asked to leave the meeting).
     1. The committee discussed confidential information submitted for this item.
  2. Part 2b – Closed session (external assessment group representatives were asked to leave the meeting)
     1. The committee then agreed on the content of the Draft Guidance (DG) or Final Draft Guidance (FDG). The committee decision was reached by consensus.
     2. The committee asked the NICE technical team to prepare the Draft Guidance (DG) or Final Draft Guidance (FDG) in line with their decisions.
* Further updates will be available on the topic webpage in due course: <https://www.nice.org.uk/guidance/indevelopment/gid-hst10044>

### Date of the next meeting

The next meeting of the Highly Specialised Technologies Evaluation Committee (HSTEC) will be held on Wednesday 12 October 2022 and will start promptly at 09.30.