NATIONAL INSTITUTE FOR HEALTH AND CARE EXCELLENCE

Centre for Health Technology Evaluation

# Highly Specialised Technologies Evaluation Committee (HSTEC) meeting minutes

**Minutes:** Confirmed

**Date:** Thursday 15 December 2022

**Location:** Via Zoom

## Attendees

Committee members present

1. Dr Peter Jackson (Chair) Present for all items
2. Dr Paul Arundel (Vice Chair) Items 5.1 to 5.2.2
3. Annett Blochberger Present for all items
4. Emtiyaz Chowdhury Present for all items
5. Sarah Davis Present for all items
6. Carrie Gardner Present for all items
7. Professor Jonathan Ives Present for all items
8. Dr Stuart Mealing Present for all items
9. Dr Mark Sheehan Present for all items
10. Karen Whitehead M.B.E Present for all items
11. Professor Ed Wilson Present for all items

NICE staff (key players) present

Richard Diaz, Associate Director Items 1.1 to 4.2.2

Jasdeep Hayre, Associate Director Items 5.1 to 5.2.2

Charlotte Bee, Programme Manager Present for all items

Daniel Davies, Project Manager Items 1.1 to 4.2.2

Celia Meyers, Project Manager Items 5.1 to 5.2.2

Rufaro Kausi, Health Technology Assessment Adviser Items 1.1 to 4.1.3

Christian Griffiths, Health Technology Assessment Adviser Items 5.1 to 5.2.2

Albany Chandler, Health Technology Assessment Analyst Items 1.1 to 4.2.2

Alan Moore, Health Technology Assessment Analyst Items 5.1 to 5.2.2

Benjamin Pearce, Senior Medical Editor Items 1.1 to 4.2.2

Emilene Coventry, Senior Medical Editor Items 5.1 to 5.2.2

Mandy Tonkinson, Public Involvement Adviser, PIP Items 1.1 to 4.1.3, Items 5 to 5.1.3

Lyn Davies, Coordinator, MIP Items 1.1 to 4.1.3, Items 5 to

5.1.3

Wajeeha Asim, Administrator, TA Items 1.1 to 4.2.2

Leah Kelly, Administrator, TA Items 5.1 to 5.2.2

Liam Murray, Administrator, COT Items 5.1 to 5.2.2

NICE staff (observers) present

Thomas Strong, Associate Director, Managed Access Items 5.1.3 to 5.2.2

Marium Uddin, Senior Manager, Managed Access Items 5 to 5.2.2

Philip Ranson, Media Relations Manager, Items 5.1.1 to 5.1.3

Stephen Norton, Technical Adviser, Managed Access Present for all items

Oyewumi Afolabi, Technical Analyst, PASLU Items 1.1 to 5.2.1

Emily Eaton Turner, Technical Analyst, Commercial Risk Assessment, Present for all items

Thomas Palmer, Technical Analyst, Managed Access Present for all items

Sarah Wilkes, Technical Analyst, Managed Access Present for all items

Milena Wobbe, Technical Analyst, Managed Access Items 1.1 to 5.2.2

Denise Moyo, Coordinator, COT Items 1.1 to 4.2.2

External assessment group representatives present

Isaac Corro Ramos, Kleijnen Systematic Reviews Items 1.1 to 4.1.3

Marie Westwood, Kleijnen Systematic Reviews Items 1.1 to 4.1.3

Abdullah Pandor, ScHARR Items 5.1.1 to 5.1.3

Paul Tappenden, ScHARR Items 5.1.1 to 5.1.3

Clinical, Patient & NHS England experts present

Dr Ayesha Ali, Medical Advisor in Highly Specialised Services, NHS England, Items 1.1 to 4.1.3, Items 5.1 to 5.1.3

Rob Burley, Patient Expert nominated by Muscular Dystrophy UK, Items 5.1 to 5.1.3

Anne-Marie Childs, Consultant Paediatric Neurologist nominated by PTC Therapeutics, Items 5.1 to 5.1.3

Helen Morris, Patient Expert nominated by Metabolic Support UK, Items 1.1 to 4.1.3

Dr Katie Moss, Clinical Expert, Consultant Rheumatologist and Honorary Senior Lecturer nominated by Alexion, Items 1.1 to 4.1.3

Francesco Mutoni, Clinical Expert, Paediatric Neurologist nominated by Muscular Dystrophy UK, Items 5.1 to 5.1.3

Dr Raja Padidela, Clinical Expert, Consultant Paediatric Endocrinology and Metabolic Bone Disorders nominated by Alexion, Items 1.1 to 4.1.3

Mark Silverman, Patient Expert, nominated by Action Duchenne, Items 5.1 to 5.1.3

Katherine Wedell, Patient Expert, nominated by Action Duchenne, Items 5.1 to 5.1.3

Melanie Williams, Patient Expert, nominated by Metabolic Support UK, Items 1.1 to 4.1.3

## Minutes

### Introduction to the meeting

* 1. The chair, Dr Peter Jackson, welcomed members of the committee and other attendees present to the meeting.
  2. The chair noted apologies from Jeremy Manuel O.B.E, Professor Philip Beales, Dr Shehla Mohammed, Stuart Davies and Anthony Williams

### News and announcements

None

### Minutes from the last meeting

The committee approved the minutes of the committee meeting held on Thursday 3 November 2022

### Evaluation of asfotase alfa for treating paediatric-onset hypophosphatasia (review of HST6) [ID3927]

* 1. Part 1 – Open session
     1. The chair welcomed the invited experts, external assessment group representatives, members of the public and company representatives from Alexion Pharma
     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.
* Emtiyaz Chowdhury declared non-financial professional and personal interests as he works for Parexel which offers manufacturers in the life science industry consultancy services. Both Alexion and PTC Therapeutics are manufacturers Parexel has worked with in the past. Emtiyaz has not been involved with any work for the technologies under appraisal at the committee meeting. It was agreed that his declaration would not prevent Emtiyaz from participating in discussions on this evaluation.
* Prior to the meeting, Dr Paul Arundel declared non-financial personal, professional, and financial interests. He is responsible for managing children with hypophosphatasia within his clinical role, leading one of three sites commissioned by NHSE to prescribe asfotase alfa and to manage children with severe hypophosphatasia in England. Accordingly, he carries local responsibility for undertaking MAA-mandated data collection. He declared receiving an honorarium fee and travel expenses from Alexion for speaking engagements previously. He also declared having recently taken over responsibility as principal investigator for the Alexion-sponsored hypophosphatasia registry study. As a clinician working in this field and the lead for one of three centres commissioned to prescribe asfotase alfa, he has had input to BPABG response to ECD from the first meeting on this topic. He attended an event at the House of Lords, ‘Celebrating 30 years of rare disease innovation and policy’, on 23 November 2022, which was supported by Alexion and Astra Zeneca. He did not receive any payment or travel expenses for this. It was agreed that these declarations would prevent Paul from participating in this section of the meeting.
* Dr Raja Padidela declared direct-financial interests as he has received research grants from Alexion for conducting clinical trials of asfotase alfa and has received honorarium and travel grants from Alexion. It was agreed that his declaration would not prevent Dr Raja from providing expert advice to the committee.
* Helen Morris declared financial interests as her organisation has received donations in the past from the Alexion which has been towards educational resources and events. It was agreed that her declaration would not prevent Helen from providing expert advice to the committee.
* Katie Moss declared a financial interest as she has received honorarium from Alexion for participating in talks. It was agreed that her declaration would not prevent Katie from providing expert advice to the committee.
* No further interests were declared for this evaluation.
  + 1. The Chair led a discussion of the consultation comments presented to the committee. This information was presented to the committee by The Chair, Peter Jackson.
  1. Part 2 – Closed session (company representatives, professional experts, external assessment group representatives and members of the public were asked to leave the meeting)
     1. The committee then agreed on the content of the Evaluation Consultation Document (ECD) or Final Evaluation Document (FED). The committee decision was reached by consensus.
     2. The committee asked the NICE technical team to prepare the Evaluation Consultation Document (ECD) or Final Evaluation Document (FED) in line with their decisions.
* Further updates will be available on the topic webpage in due course: <https://www.nice.org.uk/guidance/awaiting-development/gid-hst10046>

### 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.
* Sarah Davis declared a non-financial professional interest as she is a member of ScHARR-TAG which is acting as the EAG on this topic, but she has had no involvement with their work on this topic outside of her HST committee role. It was agreed that her declaration would not prevent Sarah from participating in discussions on this evaluation.
* Emtiyaz Chowdhury declared non-financial professional interests as he works for Parexel which offers manufacturers in the life science industry consultancy services. Both Alexion and PTC Therapeutics are manufacturers Parexel has worked with in the past (and may also be working with at present). He has not been involved with any work for the technologies under appraisal at the committee meeting. It was agreed that his declaration would not prevent Emtiyaz from participating in discussions on this evaluation.
* Dr Stuart Mealing declared non-financial professional interests as he works for YHEC which offers manufacturers in the life science industry consultancy services. Both Alexion and PTC Therapeutics are manufacturers YHEC has worked with in the past (and may also be working with at present). He has not been involved with any work for the technologies under appraisal at the committee meeting. It was agreed that his declaration would not prevent Dr Mealing from participating in discussions on this evaluation.
* Rob Burley declared a financial interest as his organisation has received funding from PTC Therapeutics. It was agreed that his declaration would not prevent Rob from providing expert advice to the committee.
* Katherine Wedell declared a personal interest as her son is taking Translarna (ataluren). It was agreed that her declaration would not prevent Katherine from providing expert advice to the committee.
* Anne-Marie Childs declared professional and 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 she was asked to present the current data from the STRIDE study to the national investigators meeting. In the last 5 years, she has also contributed to advisory boards for Roche, Biogen, and Novartis in relation to novel therapies in SMA. She 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 Anne-Marie from providing expert advice to the committee.
* Mark Silverman declared personal and professional interests as he is the parent of a child taking Translarna (ataluren). He is also a trustee of Action Duchenne and this charity has received sponsorship from PTC Therapeutics and other companies as part of its ongoing work. It was agreed that his declaration would not prevent Mark from providing expert advice to the committee.
* Professor Francesco Mutoni declared direct financial interests as he works with PTC Therapeutics and has taken part a PTC SAB held during the World Muscle Society meeting, Halifax, this year. He also took part in Stride Registry virtual meetings and was chairperson at the PTC Therapeutics EPNS Symposium this year in Glasgow. It was agreed that his declaration would not prevent Francesco from providing expert advice to the committee.
* No further interests were declared for this evaluation.
  + 1. The Chair led a discussion of the consultation comments presented to the committee. This information was presented to the committee by The Chair, Peter Jackson.
  1. Part 2 - Closed session (company representatives, professional experts, external assessment group representatives and members of the public were asked to leave the meeting)
     1. The committee then agreed on the content of the Evaluation Consultation Document (ECD) or Final Evaluation Document (FED). The committee decision was reached by consensus.
     2. The committee asked the NICE technical team to prepare the Evaluation Consultation Document (ECD) or Final Evaluation Document (FED) 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 18 January 2023 and will start promptly at 9:30am.