NATIONAL INSTITUTE FOR HEALTH AND CARE EXCELLENCE

Centre for Health Technology Evaluation

# Highly Specialised Technologies Evaluation Committee (HSTEC) meeting minutes

**Minutes:** Confirmed

**Date and time:** Wednesday 12 October 2022

**Location:** Via Zoom Conference Call

## Attendees

Committee members present

1. Dr Peter Jackson (Chair) Present for all items
2. Dr Paul Arundel (Vice Chair) Present for item 1 to 4.3
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. Jeremy Manuel O.B.E Present for all items
8. Dr Mark Sheehan Present for all items
9. Stella O’Brien Present for 1 to 4.3
10. Karen Whitehead M.B.E Present for all items
11. Tony Wootton Present for items

NICE staff present

Natalie Spray, Programme Manager Present for item 5 to 5.2.3

Charlotte Bee, Programme Manager Present for items

Richard Diaz, Associate Director Present for all items

Thomas Strong, Associate Director, Managed Access Present for item 5 to 5.2.3

Celia Mayers, Project Manager Present for item 1 to 4.3.2

Daniel Davies, Project Manager Present for item 5 to 5.2.3

Mary Hughes, Technical Adviser Present for item 1 to 4.3.2

Michelle Green, Health Technology Assessment Adviser Present for items 5 to 5.2.3

Rufaro Kausi, Health Technology Assessment Adviser Present for items 5 to 5.2.3

Tom Palmer, Health Technology Assessment Analyst Present for item 5 to 5.2.3

Stephen Norton, Health Technology Assessment Analyst Present for items 1 to 4.1.3

Anita Sangha, Technical Analyst Present for items 1 to 4.3.2

Marium Uddin, Senior Manager, Manged Access Present for items 5 to 5.2.3

Hayley Garnett, Senior Medical Editor Present for all items

Benjamin Pearce, Senior Medical Editor Present for all items

Laura Kelly, Assistant Project Manager Present for items 1 to 4.3.2

Emma Gordon, Coordinator, MiP Present for all items

Leah Kelly, Administrator, TA Present for items 1 to 4.2.3

Rumana Zaman, Administrator TA Present for item 5 to 5.2.3

External assessment group representatives present

Maiwenn Al, Kleijnen Systematic Reviews Present for items 1 to 4.2.1

Susan O’Meara, Kleijnen Systematic Reviews Present for items 1 to 4.2.1

Robert Wolff, Kleijnen Systematic Reviews Present for items 1 to 4.2.1

Issac Corro Ramos, Kleijnen Systematic Reviews Present for items 5.1 to 5.2

Marie Westwood, Kleijnen Systematic Reviews Present for items 5.1 to 5.2

Experts present

Dr Ayesha Ali, Medical Advisor in Highly Specialised Services, NHS England, Present for all items

Dr Wesley Hayes, Clinical Expert, Consultant Paediatric Nephrologist, Great Ormond Street Hospital for Children NHS Foundation Trust, Present for items 1 to 4.1.3

Dr Sally-anne Hulton, Clinical Expert, Consultant Paediatric Nephrologist, Birmingham Women's and Children's NHS Foundation Trust, Present for items 1 to 4.1.3

Dr Shabbir Moochhala, Clinical Expert, Consultant Nephrologist, Royal Free London NHS Foundation Trust, Present for items 1 to 4.1.3

Becky Shaw, Patient Expert, nominated by Metabolic Support UK, Present for items 1 to 4.1.3

Helen Morris, Patient Expert, Metabolic Support UK Present for items 5.1 to 5.2

Dr Katie Moss, Clinical Expert, Consultant Rheumatologist, St George's Hospital London, Present for items 5.1 to 5.2

Dr Raja Padidela, Clinical Expert, Consultant Paediatric Endocrinologist, Royal Manchester Children’s Hospital, Present for items 5.1 to 5.2

Melanie Williams, Patient Expert, nominated by Metabolic Support UK, Present for items 5.1 to 5.2

**Observers Present**

Kawitha Helme, Appeal Panel Member Present for items 5 to 5.2.3

Minutes

### Introduction to the meeting

* 1. The chair welcomed members of the committee and other attendees present to the meeting.

### News and announcements

* 1. None.

### Minutes from the last meeting

* 1. The committee were made aware that the minutes from the last minute will be distributed in due course and confirmed at the next meeting.

### Evaluation of Lumasiran for treating primary hyperoxaluria type 1 [ID3765]

* 1. Part 1 – Open session
     1. The chair welcomed the invited professional experts, external assessment group representatives, members of the public and company representatives from Alnylam
     2. The chair asked all committee members, professional experts, external assessment group representatives and NICE staff present to declare any relevant interests in relation to the item being considered.
* Emtiyaz Chowdhury declared a non-financial professional and personal interest, as he is an employee of Parexel which collaborates with the manufacturer. Emtiyaz does not have any knowledge of activities completed by Parexel on behalf of the manufacturer in relation to this specific technology. It was agreed that this declaration would not prevent Emtiyaz Chowdbury from participating in this section of the meeting.
* Dr Mark Sheehan declared a direct financial interest as he has received payment by Alnylam, via Clinigen (who was advising the company) for providing feedback on ethical issues in their planned Early Access to Medicines Scheme for Lumasiran. It was agreed that this declaration would not prevent Dr Sheehan from participating in this section of the meeting.
* Dr Shabbir Moochhala declared a direct financial interest as he is a Scientific advisory board member for Alnylam and Dicerna, and works in clinical private practice for renal medicine. Dr Moochhala also declared various non-financial personal and professional interests as he has worked in the following positions: Chair of NHS Rare Disease Collaborative Network for hyperoxaluria, Chair of the renal Rare Disease Group for hyperoxaluria, Steering committee member of OxalEurope, Guidelines committee member of OxalEurope, Chief Investigator for Alnylam Illuminate-C study, Principal Investigator for Alnylam BONAPH1DE Phase IV study in PH1 and Chief Investigator for 3 Dicerna trials and one trial in enteric hyperoxaluria. It was agreed that these declarations would not prevent Dr Moochala from providing expert advice to the committee.
* Dr Sally-Anne Hulton declared a direct financial interest, having received consultation fees, honoraria, and travel grant support from Alnylam and Dicerna pharmaceuticals. Dr Hulton also declared various non-financial professional and personal interests, having participated as Chief Investigator for a number of trials on behalf of Alnylam and Dicerna, and has received consultation fees and a travel grant from Chiesi (a pharmaceutical company engaged in rare disease therapies.) Dr Hulton has also been involved in the NHS Rare Disease Collaborative Network for hyperoxaluria. It was agreed that these declarations would not prevent Dr Hulton from providing expert advice to the committee.
* Dr Wesley Hayes declared an indirect financial interest,having acted as co-investigator on Illuminate clinical trials and had received travel expenses when attending the international trial investigator meeting. Dr Hayes has also been involved in the NHS Rare Disease Collaborative Network for hyperoxaluria. It was agreed that these declarations would not prevent Dr Hayes from providing expert advice to the committee.
  + 1. The Chair led a discussion of the consultation comments presented to the committee. This information was presented to the committee by the chair – Dr Peter Jackson.
  1. Part 2a – Closed session (members of the public, clinical, NHS and patient experts, and company representatives 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 Evaluation Consultation Document (ECD) or Final Evaluation Determination (FED). The committee decision was reached by consensus..
     2. The committee asked the NICE technical team to prepare the Evaluation Consultation Document (ECD) with their decisions.
* The committee asked the NICE technical team to prepare the documents in line with their decisions and further updates will be available on the topic webpage in due course: <https://www.nice.org.uk/guidance/indevelopment/gid-ta10660>.

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

* 1. Part 1 – Open session
     1. The chair welcomed the invited professional experts, external assessment group representatives, members of the public and company representatives from Alexion
     2. The chair asked all committee members, professional experts, external assessment group representatives and NICE staff present to declare any relevant interests in relation to the item being considered.
* Jeremy Manuel declared a non-financial personal interest with the chair of Metabolic Support UK. It was agreed that this declaration would not prevent Jeremy Manuel from participating in this section of the meeting.
* Dr Katie Moss declared direct and non-direct financial interests as they have received a research grant, conference sponsorship from companies, including the submitting company, and honorarium fee from Alexion. They also attend Manages Access Oversight Group Meetings as an expert representing the adult specialist centre. It was agreed that this declaration would not prevent from Katie Moss from providing expert advice to the committee.
* Dr Raja Padidela declared direct and non-direct financial interests as they have received a research grant and honorarium fees from Alexion. It was agreed that this declaration would not prevent from Raja Padidela from providing expert advice to the committee.
* Helen Morris declared a direct financial interest as the Metabolic Support UK group have received funding from the submitting company, Alexion. It was agreed that this declaration would not prevent from Helen Morris from providing expert advice to the committee.
* Prior to the meeting, Emtiyaz Chowdhury declared non-financial professional and personal interests as he is an employee of Parexel which collaborates with the manufacturer. Emtiyaz does not have any knowledge of activities completed by Parexel on behalf of the manufacturer in relation to this specific technology. It was agreed that this declaration would not prevent Emtiyaz Chowdbury from participating in this section of the meeting.
* Prior to the meeting Paul Arundel declared non-financial personal and professional, and financial interests. He is responsible for managing children with hypophosphatasia within their clinical role, leading one of three sites prescribing asfotase alfa and managing children with severe hypophosphatasia in England. Accordingly, they carry local responsibility for undertaking MAA-mandated data collection. They declared receiving an honorarium fee and travel expenses from Alexion for speaking engagements previously. They also declared having recently taken over responsibility as principal investigator for the Alexoin-sponsored hypophosphatasia registry study. It was agreed that these declarations would prevent from Paul Arundel from participating in this section of the meeting.
* Prior to the meeting, Kawitha Helme declared an indirect financial interest as they owned J&J stocks from previous employment between November 2020 until end July 2022. It was agreed that this declaration would not prevent Kawitha Helme from observing this section of the meeting.
  + 1. The Chair led a discussion of the evidence presented to the committee.. This information was presented to the committee by Annett Blochberger, Jeremy Manuel and Emtiyaz Chowdhury.
  1. Part 2 – Closed session (company representatives, clinical, patient and NHS experts, external assessment group representatives and members of the public were asked to leave the meeting).
     1. The committee discussed confidential information submitted for this item.
     2. The committee then agreed on the content of the Evaluation Consultation Document (ECD) or Final Evaluation Determination (FED). The committee decision was reached by consensus..
     3. The committee asked the NICE technical team to prepare the Evaluation Consultation Document (ECD) or Final Evaluation Determination (FED) in line with their decisions.

• The committee asked the NICE technical team to prepare the documents in line with their decisions and further updates will be available on the topic webpage in due course: <https://www.nice.org.uk/guidance/awaiting-development/gid-hst10046>

### Date of the next meeting

The next meeting of the Highly Specialised Technologies Evaluation Committee (HSTEC) will be held on Wednesday 3 November 2022 and will start promptly at 09:30am.