NATIONAL INSTITUTE FOR HEALTH AND CARE EXCELLENCE

Centre for Health Technology Evaluation

# Highly Specialised Technologies Evaluation Committee (HSTEC) meeting minutes

**Minutes:** Confirmed

**Date:** Thursday 9 February 2023

**Location:** Hybrid Meeting – 2 Redman Place, London, E20 1JQ and via Zoom

## Attendees

Committee members present

1. Dr Peter Jackson (Chair) Present for all items
2. Dr Paul Arundel (Vice Chair) 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. Carrie Gardner Present for all items
8. Tina Garvey Present for all items
9. Professor Jonathan Ives Present for all items
10. Dr Stuart Mealing Present for all items
11. Dr Shehla Mohammed Present for all items
12. Sara Payne Present for all items
13. Karen Whitehead M.B.E Present for all items
14. Professor Ed Wilson Present for all items

NICE staff (key players) present

Jasdeep Hayre, Associate Director Items 1 to 4.2.2

Richard Diaz, Associate Director Items 5.1 to 5.2.2

Celia Mayers, Project Manager Present for all items

Sally Doss, Health Technology Assessment Adviser Items 1 to 4.2.2

Alan Moore, Health Technology Assessment Analyst Items 1 to 4.2.2

Mary Hughes, Health Technology Assessment Adviser Items 5.1 to 5.2.2

Anita Sangha, Health Technology Assessment Analyst Items 5.1 to 5.2.2

Sarah Bromley, Senior Medical Editor Present for all items

Setal Bachelard, Medical Editor Present for all items

Heidi Livingston, Senior Public Involvement Adviser, PIP Items 1 to 4.1.3

Ella Fitzpatrick, Public Involvement Adviser, PIP Items 5.1 to 5.1.3

Lynn Woodward, Project Manager, Corporate Office Items 1 to 4.1.1

Lyn Davies, Coordinator, Corporate Office Items 1 to 4.1.1

Rosalee Mason, Coordinator, Corporate Office Items 1 to 5.1.3

Carl Jackson, Assistant Project Manager, COT Present for all items

Laura Kelly, Assistant Project Manager, COT Present for all items

Leah Kelly, Administrator, TA Present for all items

Liam Murray, Administrator, COT Present for all items

NICE staff (observers) present

Marium Uddin, Senior Manager, CMA Items 1 to 4.2.2

Stephen Norton, Technical Adviser, CMA Items 1 to 4.2.2

Sarah Wilkes, Technical Analyst, Commercial Liaison team Present for all items

Emily Eaton-Turner, Technical Adviser, CMA Items 5.1 to 5.2.2

Thomas Palmer, Technical Analyst, CMA Items 5.1 to 5.2.2

Kate Moore, Project Manager, TA Present for all items

External assessment group representatives present

Rebecca Bresnahan, Liverpool Reviews and Implementation Group Items 1 to 4.1.3

James Mahon, Liverpool Reviews and Implementation Group Items 1 to 4.1.3

Maiwenn Al, Kleijnen Systematic Reviews Items 5.1 to 5.1.3

Robert Wolff, Kleijnen Systematic Reviews Items 5.1 to 5.1.3

Clinical, Patient & NHS England experts present

Ayesha Ali, Medical Adviser, Highly Specialised Services – NHS England expert, Present for all items

Portia Thorman, Advocacy lead, SMA UK – patient expert nominated by SMA UK and MDUK Items 1 to 4.1.3

Ben Williams, Parent – patient expert, nominated by SMA UK and MDUK, Items 1 to 4.1.3

Laurent Servais, Professor of Paediatric Neuromuscular Diseases – clinical expert nominated by Novartis Gene Therapies Items 1 to 4.1.3

Elizabeth Wraige, Consultant Paediatric Neurologist – clinical expert nominated by Novartis Gene Therapies Items 1 to 4.1.3

Helen Morris, Individual Support Lead, Metabolic Support UK - patient expert, nominated by Metabolic Support UK Items 5.1 to 5.1.3

Sally-Anne Hulton, Consultant Paediatric Nephrologist – clinical expert, nominated by Alnylam Items 5.1 to 5.1.3

Wesley Hayes, Consultant Paediatric Nephrologist – clinical expert, nominated by British Association of Paediatric Nephrology Items 5.1 to 5.1.3

Shabbir Moochhala, Consultant Nephrologist – clinical expert, nominated by Royal Free London NHS Foundation Trust Items 5.1 to 5.1.3

## 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 Philip Beales, Anthony Williams and Jeremy Manuel.

### News and announcements

None.

### Minutes from the last meeting

The committee approved the minutes of the committee meeting held on Wednesday 18 January 2023

### Evaluation of onasemnogene abeparvovec for treating pre-symptomatic spinal muscular atrophy (MAA partial review of HST 15) [ID4051]

* 1. Part 1 – Open session
     1. The chair welcomed the invited experts, external assessment group representatives, members of the public and company representatives from Novartis Gene Therapies.
     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 a non-financial professional interest as he is an employee of Parexel who may have completed work for both manufacturers. He has not been directly involved with any work related to the technologies under consideration. It was agreed that his declaration would not prevent Emtiyaz from participating in discussions on this evaluation.
* Portia Thorman declared a financial interest as her organisation, Spinal Muscular Atrophy UK, has received funding from the industry. It was agreed that her declaration would not prevent Portia from providing expert advice to the committee.
* Elizabeth Wraige declared financial and non-financial interests as she has undertaken paid and unpaid consultancy work for Novartis and other manufacturers of spinal muscular atrophy modifying treatments. Prior to the meeting Elizabeth declared she is a member of the national SMA Reach clinical network (all paediatric neuromuscular consultants in England are members of this group) – this group meets regularly to review and discuss topics of importance with respect to standards of clinical care; She contributes data to the iSMAC registry (mandatory with respect to those patients receiving treatment through managed access agreement); She is co-chair of the national onasemnogene abeparvovec multidisciplinary team which oversees delivery of treatment through the 4 infusion centres. It was agreed that her declarations would not prevent Elizabeth from providing expert advice to the committee.
* Laurent Servais declared financial and non-financial interests as he has taken part in all trials for multiple sclerosis and he has conducted consultancy or been invited to lecture for Roche, Novartis and Biogen. He has conducted consultancy for Scholar Rock and Biohaven and he has had research funded by Novartis, Roche and Biogen. Prior to the meeting, Laurent declared he is Secretary of the world Muscle Society and vice-chair of the scientific advisory board of SMA Europe. It was agreed that his declarations would not prevent Laurent 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 The Chair.
  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 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-hst10053>

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

* 1. Part 1 – Open session
     1. The chair welcomed the invited experts, external assessment group representatives, members of the public and company representatives from Alnylam
     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 a non-financial professional interest as he is an employee of Parexel who may have completed work for both manufacturers. He has not been directly involved with any work related to the technologies under consideration. It was agreed that his declaration would not prevent Emtiyaz from participating in discussions on this evaluation.
* Dr Shabbir Moochhala declared financial and non-financial interests as he was Principal Investigator for Alnylam BONAPH1DE Phase IV study in PH1, steering committee member of OxalEurope, and guidelines committee member of OxalEurope. Prior to the meeting Dr Moochhala declared he has been scientific advisory board member for Alnylam and Dicerna, has a clinical private practice (mostly general renal medicine), has been chair of NHS Rare Disease Collaborative Network for hyperoxaluria, chair of the renal Rare Disease Group for hyperoxaluria, Chief Investigator for Alnylam Illuminate-C study and Chief Investigator for 3 Dicerna trials and one trial in enteric hyperoxaluria. It was agreed that his declarations would not prevent Dr Moochhala from providing expert advice to the committee.
* Dr Wesley Hayes declared professional interests as he is an author (with international colleagues) on some lumasiran clinical trial manuscripts. It was agreed that his declaration would not prevent Dr Hayes from providing expert advice to the committee.
* Dr Sally-Anne Hulton declared professional and financial interests as she has been Chief Investigator since 2016 for all the Alnylam studies engaged in PH1: ALN GO1, ALN GO1 002, ALN GO 003 Illuminate, ALN GO 004 Illuminate B (although her site did not recruit to this study due to Covid restrictions, she was involved with the study protocol and results review). In the role as Chief Investigator, she also acted on the safety review panel for ALN GO1. These studies were the original trials of Lumasiran (ALN GO) in healthy volunteers, followed by the studies in all patient groups in descending order of age and kidney function. She has separately received consultancy fees during the last 3 years from the company for provision of educational materials. It was agreed that her declarations would not prevent Dr Hulton from providing expert advice to the committee.
* Helen Morris declared professional interests as she works for Metabolic Support UK and works alongside the Alnylam to produce patient educational materials. It was agreed that her declarations would not prevent Helen 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.
  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-ta10660>

### Date of the next meeting

The next meeting of the Highly Specialised Technologies Evaluation Committee (HSTEC) will be held on Wednesday 15 March 2023 and will start promptly at 9:30am.