NATIONAL INSTITUTE FOR HEALTH AND CARE EXCELLENCE

Centre for Health Technology Evaluation

# Highly Specialised Technologies Evaluation Committee (HSTEC) meeting minutes

**Minutes:** Confirmed

**Date:** Thursday 10 July 2025

**Location:** Via Zoom

## Attendees

Committee members present

1. Dr Paul Arundel (Chair) Present for all items
2. Professor Iolo Doull (Vice Chair) Present for all items
3. Annett Blochberger Present for all items
4. Emtiyaz Chowdhury Present for all items
5. Carrie Gardner Present for all items
6. Tina Garvey Items 1.1 to 4.2.2
7. Professor Dusko Ilic (Observer) Items 1.1 to 5.1.3
8. Dr Natalia Kunst Items 1.1 to 4.2.2
9. Stuart Mealing Present for all items
10. Dr Shehla Mohammed Items 1.1 to 4.2.2
11. Sara Payne Items 1.1 to 4.2.2
12. Professor Ed Wilson Items 5.1 to 5.2.2
13. Richard Ballerand Items 5.1 to 5.2.2

NICE staff (key players) present

Richard Diaz, Associate Director Present for all items

Ian Watson, Associate Director Items 1.1 to 4.2.2

Jeniffer Upton, Project Manager Items 1.1 to 4.2.2

Sam Slayen, Health Technology Assessment Adviser Items 1.1 to 4.2.2

Marcela Hassova, Health Technology Assessment Analyst Items 1.1 to 4.2.2

Thomas Feist, Project Manager Items 5.1 to 5.2.2

Joanna Richardson, Health Technology Assessment Adviser Items 5.1 to 5.2.2

Ross Wilkinson, Health Technology Assessment Analyst Items 5.1 to 5.2.2

External assessment group representatives present

Hugo Peddar, University of Bristol Technology Appraisal Group, Items 1.1 to 4.1.3

Ayman Sadek, University of Bristol Technology Appraisal Group, Items 1.1 to 4.1.3

Ana Duarte, Centre for Reviews and Dissemination and Centre for Health Economics – York, Items 5.1 to 5.1.3

Clinical, Patient & NHS England experts present

Anne-Marie Childs, Consultant Paediatric Neurologist, Clinical expert nominated by ITF Pharma & Action Duchenne/ Duchenne UK, Items 1.1 to 4.1.3

Emma Hallam, Patient expert nominated by Duchenne UK, Items 1.1 to 4.1.3

Alexandra Johnson, Patient expert nominated by Duchenne UK/Action Duchenne, Items 1.1 to 4.1.3

Jack Johnson, Patient expert Nominated by Muscular Dystrophy UK, Items 1.1 to 4.1.3

Sanjeev Patel, IMF clinical lead, Clinical expert, Items 1.1 to Items 5.1.3

Dr Tracey Willis, Paediatric Neurologist, Clinical expert nominated by ITF Pharma & Muscular Dystrophy UK, Items 1.1 to 4.1.3

Liz Brownutt, Patient expert nominated by BDFA, Items 5.1 to 5.1.3

Lucy Carroll, Patient expert nominated by BDFA, Items 5.1 to 5.1.3

Paul Gissen, Paediatric Metabolic Medicine & Honorary Consultant, Clinical expert nominated by BioMarin and BDFA, Items 5.1 to 5.1.3

Gail Rich, Patient expert nominated by BDFA, Items 5.1 to 5.1.3

*Please note that alongside the attendees listed in this document, there were additional NICE Staff present in this meeting. These attendees were not involved in the decision making or discussions.*

## Minutes

### Introduction to the meeting

* 1. The chair Dr Paul Arundel welcomed members of the committee and other attendees present to the meeting.
  2. The chair noted apologies from Angharad Shambler and Jonathan Ives.

### News and announcements

* 1. The Chair acknowledged it was the last meeting for Dr Shehla Mohammed and thanked her for her service to the committee.

### Minutes from the last meeting

* 1. The committee approved the minutes of the committee meeting held on Thursday 19 June 2025.

**4. Appraisal of Givinostat for treating Duchenne muscular dystrophy in people 6 years and over [ID6323]**

Part 1 – Open session

* + 1. The chair welcomed the invited experts, external assessment group representatives, members of the public and company representatives from ITF 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. Declarations for this appraisal can be found on the Topic Register of Interest (TROI) on the topic webpage, [here](https://www.nice.org.uk/guidance/indevelopment/gid-ta11373).
    3. The Chair led a discussion of the evidence presented to the committee. This information was presented to the committee by Tina Garvey, Carrie Gardner, and Stuart Mealing.
  1. Part 2 – Closed session (company representatives, patient and clinical 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: [Project information | Givinostat for treating Duchenne muscular dystrophy in people 6 years and over [ID6323] | Guidance | NICE](https://www.nice.org.uk/guidance/indevelopment/gid-ta11373)

1. **Evaluation of Cerliponase alfa for treating neuronal ceroid lipofuscinosis type 2 (MA review of HST12) [ID6145]**
   1. Part 1 – Open session
      1. The chair welcomed the invited experts, external assessment group representatives, members of the public and company representatives from BioMarin.
      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. Declarations for this appraisal can be found on the Topic Register of Interest (TROI) on the topic webpage, [here](https://www.nice.org.uk/guidance/indevelopment/gid-hst10061).
      3. The Chair led a discussion of the consultation comments presented to the committee. This information was presented to the committee by the chair.
   2. Part 2 – Closed session (company representatives, clinical and patient 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: [Project information | Cerliponase alfa for treating neuronal ceroid lipofuscinosis type 2 (MA review of HST12) [ID6145] | Guidance | NICE](https://www.nice.org.uk/guidance/indevelopment/gid-hst10061)

### Date of the next meeting

The next meeting of the Highly Specialised Technologies Evaluation Committee (HSTEC) will be held on Thursday 21 August 2025 and will start promptly at 9:15am.