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:** Thursday 13 January 2022

**Location:** 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. Professor Ron Akehurst Present for all items
4. Sarah Davis Present for all items
5. Stuart Davies Present for all items
6. Carrie Gardner Present for all items
7. Jeremy Manuel Present for all items
8. Dr Shela Mohammed Present for all items
9. Francis Pang Present for Items 1 to 4.2.2
10. Dr Mark Sheehan Present for all items
11. Professor Lesley Stewart Present for all items
12. Karen Whitehead Present for all items

NICE staff present

Jasdeep Hayre, Associate Director Present for Items 1 to 4.2.2

Richard Diaz, Associate Director Present for Items 5 to 5.1.2

David Thomson, Associate Director Present for all items 1 to 4.2.2

Brad Groves, Associate Director Present for all items

Helen Knight, Programme Manager Present for Items 1 to 4.2.2

Gavin Kenny, Project Manager Present for Items 1 to 4.2.2

Daniel Davies, Project Manager Present for Items 5 to 5.1.2

Christian Griffiths, Health Technology Assessment Adviser Present for all items

Abi Senthinathan, Health Technology Assessment Analyst Present for all items

Catrin Austin, Technical Analyst, Methods and Economics Present for all items

Adam Brooke, Technical Analyst Present for items 5 to 5.1.2

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

Ella Livingstone, Technical Adviser, Commercial Risk Assessment Present for all items

Steve Norton, Technical Analyst, Managed access team Present for all items

Sharlene Ting, Technical Analyst Present for items 5 to 5.1.2

Heidi Livingstone, Public Involvement Adviser, PIP Present for items 1 to 4.1.3

Ismahan Abdullah, Administrator, TA Present for 1 to 4.2.2

Laura Kelly, Administrator, COT Present for all items

BMG Group representatives present

Vicky Wakefield, BMJ Group Present for items 1 to 4.1.3

Mariana Bacelar, BMJ Group Present for items 1 to 4.1.3

Experts present

Dr Ayesha Ali, Commissioning expert, NHS England Present for Items 1 to 4.1.3

Katy Brown, Patient expert, The MPS Society Present for items 1 to 4.1.3

Dr James Davidson, Clinical Expert, Great Ormond Street Hospital, Present for items 1 to 4.1.3

Dr Alex Morrison, Patient expert RDRP Present for items 1 to 4.1.3

Dr Saikat Santra, Clinical expert, Birmingham Women’s & Children’s Hospital Present for items 1 – 4.1.3

Sophie Thomas, Patient expert, The MPS Society Present for items 1 to 4.1.3

Observers present

Karen Facey, Researcher, NICE Present for all items

## Minutes

### Introduction to the meeting

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

### News and announcements

* 1. None.

### Minutes from the last meeting

* 1. The committee approved the minutes of the committee meeting held on Thursday 9 December 2021

### Evaluation of Elosulfase Alfa for treating mucopolysaccharidosis type IVa [ID1643]

* 1. Part 1 – Open session
     1. The chair welcomed the invited professional experts, external group representatives, members of the public and company representatives from BioMarin Pharmaceuticals.
     2. The chair asked all committee members, professional experts, external group representatives and NICE staff present to declare any relevant interests in relation to the item being considered.
* Jeremy Manual declared Non-Financial Professional & Personal interests, for transparency he declared that he is a board member of the Gauchers Association who are lead members of and work closely with the MPS society the patient group representing MPS patients at the LSD (Lysosomal Storage Disease) Collaborative. It was agreed that his declaration would not prevent Jeremy Manual from participating in this section of the meeting.
* Dr Paul Arundel declared a Non-Financial Professional & Personal interests as he is a local PI for trials of another drug owned/developed by BioMarin pharma (vosoritide/Voxzogo). He had no interests in direct relation to elosulfase alfa. It was agreed that his declaration would not prevent Dr Paul Arundel from participating in this section of the meeting.
* Dr James Davidson declared both a financial and non-financial interest as he is a clinician who manages patients with Morquio Syndrome and prescribed enzyme replacement therapy. He is co-author of a manuscript under preparation reporting outcomes from the managed access agreement in conjunction with other clinicians and Biomarin and of other evidence to be submitted to NICE as part of this appraisal. He received an educational grant from Biomarin to fund travel, accommodation and registration costs to attend the Lysosomal WORLD 2018 conference (February 2018). He also received an honorarium from Biomarin for attending Advisory Board on 15 March 2019 (“MPS IVa and MPS VI UK patient cohort evidence generation”).

It was agreed that his declaration would not prevent Dr James Davidson from providing expert advice to the committee.

* Dr Saikat Santra declared a financial interest as he has previously been the recipient of educational/travel grants from Biomarin and other pharmaceutical companies and is an investigator in clinical trials funded by various pharmaceutical companies including Biomarin.

It was agreed that his declaration would not prevent Dr Saikat Santra from providing expert advice to the committee.

* Alex Morrison declared an indirect interest as RDRP is a company with a number of pharmaceutical clients (including BioMarin) for whom their organisation provides a range of professional services, including clinical trial logistics and reimbursement, research and medical communications. It was agreed that her declaration would not prevent Alex Morrison from providing expert advice to the committee.
* Sophie Thomas declared an indirect financial interest as the MPS Society has received grants and funds to support the delivery of its patient services, awareness, development of its digital communications and attendance at meetings. Sophie Thomas also declared a personal interest as she has been a co-author on a number of publications and prior to 2020 had attended patient advisory boards and expert meetings. It was agreed this declaration would not prevent Sophie Thomas from participating in this discussion.
* Katy Brown declared a personal interest as trustee of the MPS society. It was agreed this declaration would not prevent Katy Brown from participating in the discussion.
  + 1. The Chair led a discussion of the consultation comments presented to the committee. This information was presented to the committee by Peter Jackson.
  1. Part 2 – Closed session (company representatives, clinical and patient experts, the NHS Commissioning expert, external group representatives and members of the public were asked to leave the meeting)
     1. The committee then agreed on the content of the Final Evaluation Determination (FED). The committee decision was reached by consensus.
     2. The committee asked the NICE technical team to prepare the documents 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-hst10027](https://www.nice.org.uk/guidance/indevelopment/gid-hst10027%20)

### Evaluation of OTL-200 for treating metachromatic leukodystrophy [ID1666]

* 1. Part 2 – Closed session
     1. The committee then agreed on the content of the Final Evaluation Determination (FED). The committee decision was reached by consensus.
     2. The committee asked the NICE technical team to prepare the Final Evaluation Determination (FED) in line with their decisions.

### Date of the next meeting

The next meeting of the Highly Specialised Technologies Evaluation Committee (HSTEC) will be held on Wednesday 16 February 2022 and will start promptly at 09:30am.