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 6 October 2021

**Location:** 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. Philip Beales Present for all items
5. Sarah Davis Present for all items
6. Stuart Davies Present for all items
7. Carrie Gardner Present for all items
8. Jeremy Manuel Present for all items
9. Dr Shehla Mohammed Present for all items
10. Francis Pang Present for all items
11. Dr Mark Sheehan Present for all items
12. Matt Smith Present for all items
13. Professor Lesley Stewart Present for items 1 to 5
14. Karen Samuels Present for items 2 to 7

NICE staff present

Nicole Elliott, Associate Director Present for items 5 to 7

Helen Knight, Programme Manager Present for items 1 to 5

Jasdeep Hayre, Associate Director Present for items 1 to 5

Gavin Kenny, Project Manager Present for items 1 to 5

Ismahan Abdullah, Administrator Present for items 1 to 5

Jeremy Powell, Project Manager Present for items 5 to 7

Sophie McHugh, Administrator Present for all items

Gemma Smith, Coordinator Present for items 5 to 6

Daniel Greenwood, Assistant Administrator Present for notes 5 to 7

James Devine, Coordinator Items 1 to 5

Heidi Livingstone, Public involvement adviser Items 1 to 4.2 & 5 to 5.2

Helen Barnett, Senior Medical Editor Items 1 to 5

Abitha Senthinathan, Technical Analyst Items 1 to 5

Christian Griffiths, Analyst - technology appraisal Items 1 to 5

Adam Brooke, Technology Analyst Items 5 to 6

Sharlene Ting, Technical Analyst Items 5 to 6

Sandra Robinson, Assistant Project Manager Items 1 to 4.2 & 5 to 5.2

Lyn Davies, Coordinator Items 1 to 4.2 & 5 to 5.2

Catherine Pank, Assistant Project Manager Items 5 to 7

Ella Livingstone, Technical adviser - Commercial Risk Assessment Present for all items

Claire Hawksworth, Technical Analyst – Evidence Generation Present for all items

Emily EatonTurner, Technical analyst - Commercial risk assessment Items 1 to 5

Brad Groves, Associate Director - Managed Access Present for all items

Charlotte Bee, Programme Manager (Data Projects) Items 1 to 5

External group representatives present

Vicky Wakefield, BMJ Items 1 to 4.2

Marina Bacelar, BMJ Items 1 to 4.2

Matthew Walton, York TAR Items 5 to 5.2

Warwick Evidence Items to 5 to 5.2

Rob Hodgson, York TAR Items 5 to 5.2

**Experts present**

Ayesha Ali, Commissioning Expert, NHSE Items 1 to 4.2 and 5

Dr James Davison, Clinical Expert, Great Ormond Street Hospital, Items 1 to 4.2 & 5 to 5.2

Dr Elaine Murphy, Clinical expert, UCL NHS Foundation Trust and BIMD, Items 1 to 4.2

Dr Saikat Santra, Clinical expert, Birmingham Women’s & Children’s Hospital, Items 1 to 4.2

Sophie Thomas, Patient expert, The MPS Society Items 1 to 4.2 & 5 to 5.2

Alex Morrison, Patient expert RDRP Items 1 to 4.2

Katy Brown, Patient expert, The MPS Society Items 1 to 4.2

Georgina Morton, Founder, ArchAngle MLD Items 5 to 5.2

Nicole Elson, Patient Expert, ArchAngle MLD Items 5 to 5.2

Francesca Fumagalli, Clinical expert, San Raffaele Hospital Items 5 to 5.2

## 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 approved the minutes of the committee meeting held on Wednesday 15 September 2021.

### Evaluation of Elosulfase alfa for treating mucopolysaccharidosis type Iva (re-evaluation of HST2) (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 a 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 interest 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 Elaine Murphy declared a Direct – non-financial interest as she is an investigator for the following study: A Multicenter, Multinational, Observational Morquio a Registry Study (MARS)
* It was agreed that her declaration would not prevent Dr Elaine Murphy 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 we provide 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.
  + 1. The Chair led a discussion of the evidence presented to the committee. This information was presented to the committee by the lead team, Shehla Mohammed, Lesley Stewart & Mark Sheehan.
  1. Part 2 - Closed session (company representatives, professional experts, external group representatives and members of the public were asked to leave the meeting)
     1. 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-hst10027>

### Evaluation of OTL-200 for treating metachromatic leukodystrophy (ID1666)

* 1. Part 1 – Open session
     1. The chair welcomed the invited professional experts, external group representatives, members of the public and company representatives from Orchard Therapeutics.
     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.
* Georgina Morton declared an indirect financial interest as she ArchAngel MLD Trust received a grant of £5000 in December 2019 from Orchard Therapeutics in support of work towards newborn screening for MLD and ArchAngel MLD Trust, The MPS Society and MLD Support Association UK have jointly received a grant of £11,600 in August 2020 to carry out an MLD patient and caregiver burden survey.
* It was agreed that her declaration would not prevent Georgina Morton from providing expert advice to the committee.
* Dr James Davidson declared a financial interest as he has provided remunerated consultancy (Advisory Board member) to Orchard Therapeutics.
* It was agreed that his declaration would not prevent Dr James Davidson from providing expert advice to the committee.
  + 1. The Chair led a discussion of the evidence presented to the committee.
  1. Part 2 – Closed session (company representatives, professional experts, external group representatives and members of the public were asked to leave the meeting).
     1. 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-hst10028> .

### Date of the next meeting

The next meeting of the Highly Specialised Technologies Evaluation Committee (HSTEC) will be held on Wednesday 10 November 2021 and will start promptly at 9:00am.