NATIONAL INSTITUTE FOR HEALTH AND CARE EXCELLENCE

Centre for Health Technology Evaluation

Highly Specialised Technologies Evaluation Committee (HSTEC) meeting minutes

**Minutes:** Unconfirmed

**Date and time:** Thursday 13 May 2021, 9:30am to 16:30pm

**Location:** Zoom

# 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 items 5 to 6
4. Mr Sotiris Antoniou Present for all items
5. Professor Philip Beales Present for all items
6. Ms Sarah Davis Present for all items
7. Stuart Davies Present for all items
8. Mrs Carrie Gardner Present for all items
9. Jeremy Manuel Present for all items
10. Dr Shehla Mohammed Present for all items
11. Francis Pang Present for items 1 to 4.2.3
12. Dr Mark Sheehan Present for all items
13. Professor Lesley Stewart Present for all items

# NICE staff present:

Nicole Elliott, Associate Director Present for items 5 to 6

Jasdeep Hayre, Associate Director Present for items 2 to 5

Jo Ekeledo, Project Manager Present for items 5 to 6

Gavin Kenny, Project Manager Present for items 2 to 5

Ismahan Abdullah, Administrator Present for items 2 to 5

Mira Patel, COT Coordinator Present for items 2 to 5

Daniel Greenwood, COT Assistant Administrator Present for items 5 to 6

Sally Doss, Health Technology Assessment Adviser Present for items 2 to 5

Verena Wolfram, Assessment Analyst Present for items 2 to 5

Lucinda Evans, Coordinator, MIP Present for items 2 to 5

Heidi Livingstone, Senior Public Involvement Adviser Present for items 2 to 3.2

Ann Greenwood, Senior medical editor Present for items 2 to 5

Helen Barnett, Editor Present for items to 4 to 5

Emily Eaton Turner, HTA Adviser Present for items 3.1.3 to

Phil Ranson, Comms Lead Present for items 4 to 5

Nigel Gumbleton, HTA Analyst Present for items 2 to 5

# External group representatives present:

Professor G.J Melendez-Torres, Director, PenTAG Present for items 2 to 4.2

Mr Brian O’Toole, Health Economist, PenTAG Present for items 2 to 4.2

# Professional experts present:

Professor Mike Badminton, Clinical Expert, Global Porphyria Present for items 2 to 4.2  
Advocacy Coalition & National Acute Porphyria Service

Mr Salman Fatmi, Patient Expert, Present for items 2 to 4.2  
British Porphyria Association,

Mrs Liz Gill, Patient Expert, British Porphyria Association, Present for items 2 to 4.2

Ms Fiona Marley, Commissioning Expert, NHSE, Present for items 2 to 4.2

Dr Penelope Stein, Clinical Expert, The British Porphyria Present for items 2 to 4.2  
Association & Alnylam Pharmaceuticals,

1. Minutes from the last meeting
   1. The committee approved the minutes of the committee meeting held on Thursday 15 April 2021 in a private session.
2. Introduction to the meeting
   1. The chair welcomed members of the committee and other attendees present to the meeting.
   2. The chair noted apologies from Dr Matt Smith.
3. Evaluation of Givosiran for treating acute hepatic porphyria [ID1549]
   1. Part 1 – Open session
      1. The chair welcomed the invited professional experts, external group representatives, members of the public and company representatives from Alnylam 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.

* Prior to the meeting, Professor Ron Akehurst, declared direct financial interests as the company of which he is Chairman, is providing consultancy advice to the manufacturer of Givosiran, Alnylam Pharmaceuticals. It was agreed that this declaration would prevent Professor Akehurst from participating in this section of the meeting and he was asked to leave for the duration of the item.
* Dr Mark Sheehan, committee member, declared a direct financial interest as he undertook paid work for Alnylam Pharmaceuticals within the last 12 months. The work was in an unrelated disease area. It was agreed that this declaration would not prevent Dr Sheehan from participating in this section of the meeting.
* Dr Penelope Stein, clinical expert, declared direct non-financial interests as she has been actively involved (sub-investigator) in clinical trials of givosiran at King’s College Hospital since 2016. Prior to the meeting, Dr Stein declared direct financial interests as she received funds from Alnylam Pharmaceuticals to attend an international porphyria meeting in 2018. It was agreed that these declarations would not prevent Dr Stein from providing expert advice to the committee.
* Prior to the meeting, Professor Mark Badminton, clinical expert, declared indirect interests as he has been actively involved in a longitudinal study of severe acute porphyria patients funded by Alnylam Pharmaceuticals and is also on the Scientific Advisory Board for the ELEVATE post marketing givosiran registry. It was agreed that these declarations did not prevent Professor Badminton from providing expert advice to the committee.
* No further conflicts of interest were declared for this item.
  + 1. The Chair then introduced the lead team, Dr Shehla Mohammed, Mr Stuart Davies and Dr Mark Sheehan, who gave presentations on the clinical effectiveness and cost effectiveness of givosiran for treating acute hepatic porphyria [ID1549].
  1. Part 2 – Closed session (company representatives, professional experts, external group representatives and members of the public were asked to leave the meeting 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). The committee decision was reached by consensus.
     3. The committee asked the NICE technical team to prepare the Evaluation Consultation Document (ECD) in line with their decisions.

1. Evaluation of Onasemnogene abeparvovec for treating spinal muscular atrophy [ID1473]
   1. Part 1 – Open session
      1. The chair welcomed the invited professional experts and members of the public and company representatives from Novartis Gene Therapy.
      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.

* Prior to the meeting, Mr Francis Pang, committee member, declared a direct financial interest as Francis is a former employee and shareholder of one of the comparator companies: Biogen. It was agreed that this declaration would prevent Mr Pang from participating in this section of the meeting and he was asked to leave for the duration of the item.
* No further conflicts were declared for this topic.
  + 1. The Chair introduced the key themes arising from the consultation responses to the Evaluation Consultation Document [ECD] received from consultees, commentators and through the NICE website.
  1. Part 2a – Closed session (members of the public were asked to leave the meeting).
     1. The committee discussed confidential information submitted for this item.
  2. Part 2b – Closed session (company representatives, professional experts, external 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 Appraisal Determination [FAD]. The committee decision was reached by consensus.
     2. The committee asked the NICE technical team to prepare the Appraisal Consultation Document (ACD) or Final Appraisal Determination [FAD] in line with their decisions.

1. Date of the next meeting

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