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 3 November 2022, 09:30am

**Location:** Via Zoom

## Attendees

Committee members present

1. Dr Peter Jackson Present for all items
2. Dr Paul Arundel (Chair) Present for all items
3. Professor Ron Akehurst Present for all items
4. Professor Philip Beales Present for all items
5. Annett Blochberger Present for all items
6. Emtiyaz Chowdhury Present for all items
7. Stuart Davies Present for all items
8. Sarah Davis Present for all items
9. Carrie Gardner Present for all items
10. Jeremy Manuel O.B.E Present for all items
11. Dr Stuart Mealing Present for all items
12. Dr Mark Sheehan Present for all items
13. Karen Whitehead M.B.E Present for all items
14. Anthony Williams Present for all items

NICE staff present

Jasdeep Hayre, Associate Director Present for all items

Celia Mayers, Project Manager Present for all items

Christian Griffiths, Heath Technology Assessment Adviser Present for all items

Stephen Norton, Technical Adviser, Managed Access Items 3.1.3 to 3.3.2

Luke Cowie, Heath Technology Assessment Analyst Present for all items

Oyewumi Afolabi, Technical Analyst Present for all items

Catrin Austin, Technical Analyst Present for all items

Owen Harrison, Heath Technology Assessment Analyst Items 1 to 3.1.3

Thomas Palmer, Heath Technology Assessment Analyst Present for all items

Sarah Wilkes, Technical Analyst Present for all items

Milena Wobbe, Technical Analyst Present for all items

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

Neha Jiandani, Technical Analyst, Commercial Liaison Present for all items

Helen Barnett, Senior Medical Editor Present for all items

Mandy Tonkinson, Public Involvement Adviser, PIP Items 1 to 3.1.3

Rosalee Mason, Coordinator, MIP Items 1 to 3.1.3

Liam Murray, Administrator, COT Present for all items

Ayla Hudson, Apprentice, Planning & Ops Items 1 to 3.1.3

**External assessment group representatives present**

Neelam Kalita, Southampton Health Technology Assessments Centre Items 1 to 3.2

Joanne Lord, Southampton Health Technology Assessments Centre Items 1 to 3.2

Clinical, NHS and Patient experts present

Dr Ayesha Ali, Medical Advisor in Highly Specialised Services, NHS England, Present for all items

Jess Doyle, Head of Insight – Patient Expert, Metabolic Support UK, items 1 to 3.1.3

Professor Simon Heales, Founder and Trustee – Clinical Expert, The AADC Research Trust, items 1 to 3.1.3

Professor Manju Kurian, NIHR Research Professor, UCL Professor of Neurogenetics and Honorary Consultant in Paediatric Neurology, nominated by The AADC Research Trust, items 1 to 3.1.3

Richard Poulin, Patient Expert, nominated by Metabolic Support UK , items 1 to 3.1.3

Julie Ramsay, Charity Manager - Patient Expert, The AADC Research Trust, items 1 to 3.1.3

## Minutes

### 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 Shehla Mohammed and Professor Ed Wilson.

### News and announcements

* 1. None.

### Evaluation of Evaluation of eladocagene exuparvovec for treating aromatic L-amino acid decarboxylase deficiency [ID3791]

* 1. Part 1 – Open session
     1. The chair - Dr Paul Arundel - welcomed the invited professional experts, external assessment group representatives, members of the public and company representatives from PTC Therapeutics Limited
     2. The chair asked all committee members, professional 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 and personal interest as his employer - Parexel - is a CRO which works with PTC. Neither Emtiyaz or Parexel has have not been involved in any consulting activities for this specific technology. It was agreed that this declaration would not prevent Emtiyaz Chowdhury from taking part in this discussion.
* Dr Stuart Mealing declared a non-financial professional and personal interest as his employer – YHEC - has undertaken work for PTC in relation to this molecule in the recent past. He has never personally worked on this, or for the company. It was agreed that this declaration would not prevent Dr Mealing from taking part in this discussion
* Jess Doyle declared an indirect financial interest as her employer – Metabolic Support UK has received funding from PTC towards core programmes and their annual event. It was agreed that this declaration would not prevent Jess Doyle from providing expert advice to the committee.
* Professor Manju Kurian declared various non-financial professional and personal interests, as his employer - Great Ormond Street - have been in discussions with PTC about bidding to be one of the clinical sites for this gene therapy, and Professor Kurian was part of the team involved the discussions. Professor Kurian has also worked with Professor Krys Bankiewicz on an alternative gene therapy approach using a similar viral vector but different brain target. This work has been undertaken on a research basis, but his team has not received any financial renumeration from PTC for involvement in this research. Furthermore, Professor Kurian also declared her position on the medical and scientific advisory board for the AADC research trust. It was agreed that these declarations would not prevent Professor Kurian from providing expert advice to the committee.
* Richard Poulin declared financial and non-financial interests having received non-paid benefits and renumeration for speaking engagements from PTC. The speaking engagements were regarding parent experience regarding misdiagnosis, genetic testing experience, and symptoms related to aromatic l-amino acid decarboxylase deficiency, however, Richard Poulin is not receiving any payments from PTC for activities in the UK or related to the eladocagene exuparvovec NICE appraisal. It was agreed that these declarations would not prevent Richard Poulin from providing expert advice to the committee.
* Professor Simon Heales declared a financial interest as he has received speaker fees and an unrestricted grant from PTC. It was agreed that this declaration would not prevent Professor Heales 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 – Dr Carrie Gardner (Clinical), Stuart Davies (Cost) and Dr Mark Sheehan (Lay).
  1. Part 2a – Closed session (company representatives, clinical and patient experts and 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 (external assessment group representatives 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) in line with their decisions
* 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-hst10052>

### Date of the next meeting

The next meeting of the Highly Specialised Technologies Evaluation Committee (HSTEC) will be held on Thursday 15 December 2022 and will start promptly at 09:30am.