

## Highly Specialised Technologies Evaluation Committee Meeting

**Minutes:** Confirmed

**Date and Time:** Tuesday 17 November 2015, 10.00am to 5.00pm

**Venue:** National Institute for Health and Care Excellence  
Level 1A, City Tower  
Piccadilly Plaza  
Manchester  
M1 4BT

<b>Present:</b>		
1.	Dr Peter Jackson (Chair)	Present for all notes
2.	Prof Ron Akehurst	Present for all notes
3.	Dr Jonathan Howell	Present for all notes
4.	Jeremy Manuel	Present for all notes
5.	Sarah Davis	Present for all notes
6.	Linn Phipps	Present for all notes
7.	Dr Mark Sheehan	Present for all notes
8.	Prof Lesley Stewart	Present for all notes
9.	Dr Trevor Cole	Present for all notes
10.	Dr Vincent Kirkbride	Present for all notes

### In attendance:

Meindert Boysen	Programme Director, National Institute for Health and Care Excellence	Present for all notes
Linda Landells	Technical Adviser, National Institute for Health and Care Excellence	Present for all notes
Pilar Pinilla-Dominguez	Technical Analyst National Institute for Health and Care Excellence	Present for all notes
Leanne Wakefield	Project Manager, National Institute for Health and Clinical Excellence	Present for all notes
Prof Aileen Clarke	Warwick Evidence	Present for notes 1 to 17
Dr Joshua Pink	Warwick Evidence	Present for notes 1 to 17
Stuart W.Peltz	PTC Therapeutics	Present for notes 1 to 17

Jade Marshall	PTC Therapeutics	Present for notes 1 to 17
Dr Michela Guglieri	Clinical Specialist	Present for notes 1 to 17
Dr Adnan Manzur	Clinical Specialist	Present for notes 1 to 17
Gary Hill	Patient Expert	Present for notes 1 to 17
Robert Meadowcroft	Patient Expert	Present for notes 1 to 17
Bernie Mooney	Patient Expert	Present for notes 1 to 17
Dr Edmund Jessop	NHS England	Present for notes 1 to 17

### **Non-public observers:**

Helen Barnett	Editor	Present for all notes
Chloe Kastoryano	Public Involvement Adviser	Present for all notes
Jenna Dilkes	Highly Specialised Technologies	Present for all notes
Paula O'Grady	Technology Appraisals	Present for notes 1 to 15

### **Notes**

#### **Welcome**

1. The Chair welcomed all members of the Committee and other attendees present to the meeting. The Chair reviewed the agenda and timescales for the meeting, which included the Evaluation of ataluren for treating Duchenne muscular dystrophy caused by a nonsense mutation in the dystrophin gene
2. The Chair informed the Committee of the non-public observers at this meeting: Helen Barnett, Chloe Kastoryano, Jenna Dilkes and Paula O'Grady.
3. Apologies were received from Sotiris Antoniou, Steve Brennan and Dr Anthony Wierzbicki and Francis Pang

#### **Any other Business**

4. Committee Members received an update on current evaluations for highly specialised technologies.

#### **Notes from the last meeting**

5. The minutes from the meeting which took place on 21 October 2015 were agreed.

## **Evaluation of ataluren for treating Duchenne muscular dystrophy with a nonsense mutation in the dystrophin gene**

### **Part 1 – Open session**

6. The Chair welcomed the invited experts: Dr Michela Guglieri, Dr Adnan Manzur, Gary Hill, Robert Meadowcroft and Bernie Mooney to the meeting and they introduced themselves to the Committee.
7. The Chair welcomed representatives from PTC Therapeutics to the meeting.
8. The Chair asked all Committee members to declare any relevant interests
  - 8.1. Dr Peter Jackson, Prof Ron Akehurst, Dr Trevor Cole, Sarah Davis, Dr Jonathan Howell, Dr Vincent Kirkbride, Jeremy Manuel, Dr Mark Sheehan and Prof Lesley Stewart, all declared that they knew of no personal specific pecuniary interest, personal non-specific pecuniary interest, non-personal specific pecuniary interest, non-personal non-specific pecuniary interest, personal specific family interest or personal non-specific family interest for any of the technologies to be considered as part of the evaluation of ataluren for treating Duchenne muscular dystrophy caused by a nonsense mutation in the dystrophin gene
  - 8.2. Linn Phipps declared a personal non-pecuniary interest due to her role as Patient & Public Voice Representative on NHS England's CPAG (Clinical Priorities Advisory Group)
    - 8.2.1. It was agreed that this declaration would not prevent Linn Phipps from participating in the meeting
9. The Chair asked all NICE Staff to declare any relevant interests.
  - 9.1. All declared that they knew of no personal specific pecuniary interest, personal non-specific pecuniary interest, non-personal specific pecuniary interest, non-personal non-specific pecuniary interest, personal specific family interest or personal non-specific family interest for any of the technologies to be considered as part of the evaluation of ataluren for treating Duchenne muscular dystrophy caused by a nonsense mutation in the dystrophin gene
  - 9.2. Sheela Upadhyaya (Associate Director) declared a personal pecuniary interest due to her participation in an advisory board with PTC Therapeutics in February 2015
    - 9.2.1. It was agreed that this declaration would not prevent Sheela Upadhyaya from participating in the meeting itself, but would prevent her participating in the event of a vote by Committee Members
10. The Chair asked all other invited guests assessment group/ERG and invited experts, not including observers) to declare their relevant interests.
  - 10.1. All declared that they knew of no personal specific pecuniary interest, personal non-specific pecuniary interest, non-personal specific pecuniary interest, non-personal non-specific pecuniary interest, personal specific family interest or personal non-specific family interest for any of the

technologies to be considered as part of the evaluation of ataluren for treating duchenne muscular dystrophy caused by a nonsense mutation in the dystrophin gene

11. The Committee then discussed the responses from the consultation, the clinical effectiveness, patient perspective and cost effectiveness of **ataluren for treating Duchenne muscular dystrophy with a nonsense mutation in the dystrophin gene** on the basis of the evidence before them. The discussions included:
12.
  - 12.1. The response from the company to the Committee's request for additional information on the first evaluation consultation document and the Evidence Review Group's critique of this additional information. The response included the results of the multicentre, randomised, double-blind, placebo-controlled confirmatory study (PTC124-GD-020-DMD; Study 020) and an updated economic analysis incorporating results from Study 020 and different assumptions.
  - 12.2. The nature of the condition and the impact that ataluren might have on patients and caregivers' quality of life.
  - 12.3. The benefits of ataluren in comparison with its associated cost to the NHS and Personal Social Services. The cost of ataluren were discussed in the context of the costs incurred by the company for research, development and manufacturing. The costs and benefits of ataluren were compared with those for other treatments for small populations.
  - 12.4. Whether ataluren represents good value for money to the NHS in light of the benefits that it provides.
13. The Chair asked the company representatives whether they wished to comment on any matters of factual accuracy.
14. The Chair explained that "representatives of the press and other members of the public be excluded from the remainder of this meeting having regard to the confidential nature of the business to be transacted, publicity on which would be prejudicial to the public interest" (Section 1(2) Public Bodies (Admission to Meetings) Act 1960)" and all public attendees left the meeting.
15. The Chair then thanked the experts and company representatives for their attendance, participation and contribution to the evaluation and they left the meeting.

### **Part 2a – Closed session**

16. The company representatives and experts made closing statements and answered the Committee's questions regarding confidential information that could not be discussed during the public part of the meeting.
17. The Chair then thanked the experts and company representatives for their attendance, participation and contribution to the evaluation and they left the meeting.

### **Part 2b – Closed session**

18. Discussion on confidential information continued.

19. The Committee instructed the technical team to prepare the Evaluation Consultation Document (ECD) in line with their decisions.

**Date, time and venue of the next meeting**

20. Wednesday 20 January 2016 at 10.00am at National Institute for Health and Care Excellence, Level 1A, City Tower, Piccadilly Plaza, Manchester M1 4BT.