

Highly Specialised Technologies Evaluation Committee Meeting

Minutes: **Confirmed**

Date and Time: Wednesday 17 February 2016, 10am to 2pm

Venue: Friend's Meeting House
6 Mount Street
Manchester
M2 5NS

Present:	1. Peter Jackson, Chair 2. Ron Akehurst 3. Sotiris Antoniou 4. Sarah Davis 5. Jonathan Howell 6. Vincent Kirkbride 7. Jeremy Manuel 8. Mark Sheehan 9. Lesley Stewart 10. Anthony Wierzbicki	Present for all notes Present for all notes
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In attendance:

Meindert Boysen	Programme Director, National Institute for Health and Care Excellence	Present for all notes
Sheela Upadhyaya	Associate Director, National Institute for Health and Care Excellence	Present for all notes
Jenna Dilkes	Project Manager, National Institute for Health and Care Excellence	Present for all notes
Leanne Wakefield	Project Manager, National Institute for Health and Care Excellence	Present for all notes
Linda Landells	Technical Adviser, National Institute for Health and Clinical Excellence	Present for all notes
Janis Clayton	PTC Therapeutics	Present for notes 1 to 16

Marcio Souza	PTC Therapeutics	Present for notes 1 to 16
Michela Guglieri	Clinical Expert	Present for notes 1 to 14
Adnan Manzur	Clinical Expert	Present for notes 1 to 14
Gary Hill	Patient Expert	Present for notes 1 to 14
Robert Meadowcroft	Patient Expert	Present for notes 1 to 14
Bernie Mooney	Patient Expert	Present for notes 1 to 14

Non-public observers:

Helen Barnett	Editor, NICE	Present for all notes
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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 evaluations 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.
3. Apologies were received from Steve Brennan, Trevor Cole, Francis Pang and Linn Phipps.

Any other Business

4. NONE

Notes from the last meeting

5. The minutes from the meeting which took place on the 20 January 2016 were agreed.

Evaluation of ataluren for treating Duchenne muscular dystrophy caused by a nonsense mutation in the dystrophin gene

Part 1 – Open session

6. The Chair welcomed the invited experts: Janis Clayton, Marcio Souza, Michela Guglieri, Adnan Manzur, Gary Hill, Robert Meadowcroft and Bernie Mooney to the meeting and they introduced themselves to the Committee.
7. The Chair welcomed company representatives from PTC Therapeutics to the meeting.
8. The Chair asked all Committee members to declare any relevant interests
 - 8.1. Peter Jackson, Ron Akehurst, Sotiris Antoniou, Sarah Davis, Jonathan Howell, Vincent Kirkbride, Jeremy Manuel, Mark Sheehan and Lesley Stewart all declared that they knew of no personal specific financial interest, personal non-specific financial interest, non-personal specific financial interest, non-personal non-specific financial 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 Duchenne muscular dystrophy caused by a nonsense mutation in the dystrophin gene

8.2. Anthony Wierzbicki declared a non-personal non-specific financial interest due to his role as the site clinical trial investigator for lipid-lowering drug therapies for Amgen and Merck Sharp & Dohme. Also site lead for clinical registry for Chiesi.

8.2.1. It was agreed that this declaration would not prevent Anthony Wierzbicki from participating in the meeting.

1. The Chair asked all NICE Staff to declare any relevant interests.
 - 1.1. All declared that they knew of no personal specific financial interest, personal non-specific financial interest, non-personal specific financial interest, non-personal non-specific financial 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.
2. The Chair asked all other invited guests (assessment group/ERG and invited experts, not including observers) to declare their relevant interests.
 - 2.1. All declared that they knew of no personal specific financial interest, personal non-specific financial interest, non-personal specific financial interest, non-personal non-specific financial 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.
3. The Chair introduced the key themes arising from the recent discussions regarding a Managed Access Agreement.
4. The Chair asked the company representatives whether they wished to comment on any matters of factual accuracy.
5. 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.
6. 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

7. Discussion on confidential information continued. This information was supplied by the company.

8. The Chair then thanked the Company representatives for their attendance, participation and contribution to the evaluation and they left the meeting.

Part 2b – Closed session

9. Discussion on confidential information continued.
10. The Committee instructed the technical team to prepare the Final Evaluation Determination (FED) in line with their decisions.

Date, time and venue of the next meeting

11. Tuesday 22 March 2016 at National Institute for Health and Care Excellence, Level 1A, City Tower, Piccadilly Plaza, Manchester M1 4BT.