

Highly Specialised Technologies Evaluation Committee Meeting

Minutes: Unconfirmed

Date and Time: Thursday 17 September 2015, 10.00am to 5.00pm

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

Present:	1. Dr Peter Jackson (Chair)	Present for all notes
	2. Prof Ron Akehurst	Present for all notes
	3. Steve Brennan	Present for all notes
	4. Dr Jonathan Howell	Present for all notes
	5. Jeremy Manuel	Present for all notes
	6. Sarah Davis	Present for all notes
	7. Linn Phipps	Present for all notes
	8. Dr Mark Sheehan	Present for all notes
	9. Prof Lesley Stewart	Present for all notes
	10. Dr Trevor Cole	Present for all notes
	11. Sheela Upadhyaya	Present for all notes
	12. 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
Victoria Kelly	Technical Analyst, 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	Present for all notes

Excellence

Prof Aileen Clarke	Warwick Evidence	Present for notes 1 to 16
Dr Joshua Pink	Warwick Evidence	Present for notes 1 to 16
Stuart W.Peltz	PTC Therapeutics	Present for notes 1 to 16
Jade Marshall	PTC Therapeutics	Present for notes 1 to 16
Dr Michela Guglieri	Clinical Specialist	Present for notes 1 to 16
Dr Adnan Manzur	Clinical Specialist	Present for notes 1 to 16
Gary Hill	Patient Expert	Present for notes 1 to 16
Robert Meadowcroft	Patient Expert	Present for notes 1 to 16
Bernie Mooney	Patient Expert	Present for notes 1 to 16
Dr Edmund Jessop	NHS England	Present for notes 1 to 16

Non-public observers:

Helen Barnett	Editor	Present for all notes
Chloe Kastoryano	Public Involvement Adviser	Present for all notes
Ailish Higgins	Scientific Advice	Present for all notes

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 and Ailish Higgins.
3. Apologies were received from Dr Antony 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 July 2015 were agreed, with the addition of the declaration of interest for Linn Phipps (Due to her role as Patient & Public Voice Representative on NHS England's CPAG (Clinical Priorities Advisory Group))

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 Guglieru, Dr Adnan Manzur, Gary Hill, Robert Meadowcroft and Bernie Mooney to the meeting and they introduced themselves to the Committee.
7. The Chair welcomed c 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, Steve Brennan, Dr Jonathan Howell, Jeremy Manuel, Sarah Davis, Dr Mark Sheehan, Prof Lesley Stewart, Dr Trevor Cole and Dr Vincent Kirkbride 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.
 - 8.3. Sheela Upadhyaya declared a personal pecuniary interest due to her participation in an advisory board with PTC Therapeutics in February 2015
 - 8.3.1 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
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

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 Chair introduced the lead team, Dr Jonathan Howell , Dr Mark Sheehan and Prof Ron Akehurst who gave presentations on the clinical effectiveness, patient perspective and cost effectiveness of **ataluren for treating Duchenne muscular dystrophy with a nonsense mutation in the dystrophin gene**.
12. The Committee then discussed 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:
13.
 - 13.1. Whether the outcomes assessed in Study 007 provide suitable information to adequately assess the benefits associated with ataluren
 - 13.2. The clinical significance of the treatment effects.
 - 13.3. Whether the Committee consider the company's post hoc subgroup analyses of patients in the decline phase to be suitable for informing its decision-making?
 - 13.4. Whether the Committee have enough information to inform its conclusions on the rate of serious adverse events expected with ataluren treatment
 - 13.5. Is the clinical effectiveness of ataluren and best supportive care appropriately modelled?
 - 13.6. What are the Committee's preferred assumptions for the economic model?
 - 13.7. Whether the company's model appropriately captured the costs and consequences associated with ataluren?
 - 13.8. Whether the company's budget impact analysis was reasonable
 - 13.9. Whether there are any significant benefits of ataluren, beyond direct health benefits, which have not been taken into account in the economic analysis
14. The Chair asked the company representatives whether they wished to comment on any matters of factual accuracy.
15. 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.

16. The Chair then thanked the experts and company representatives for their attendance, participation and contribution to the evaluation and they left the meeting.

Part 2 – Closed session

17. Discussion on confidential information continued.
18. 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

19. Wednesday 21 October 2015 at 10.00am at National Institute for Health and Care Excellence, Level 1A, City Tower, Piccadilly Plaza, Manchester M1 4BT.