

Highly Specialised Technologies Evaluation Committee Meeting

Minutes: Confirmed

Date and Time: Wednesday 25 April 2018

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

Present:	1. Dr Peter Jackson	Present for all notes
	2. Prof Ron Akehurst	Present for all notes
	3. Sotiris Antoniou	Present for all notes
	4. Dr Ayesha Ali	Present for all notes
	5. Sarah Davis	Present for all notes
	6. Stuart Davies	Present for all notes
	7. Carrie Gardner	Present for all notes
	8. Jeremy Manual	Present for all notes
	9. Francis Pang	Present for all notes
	10. Linn Phipps	Present for all notes
	11. Dr Mark Sheehan	Present for all notes
	12. Professor Lesley Stewart	Present for all notes

In attendance:

Meindert Boysen	Programme Director, National Institute for Health and Care Excellence	Present for all notes
Helen Knight	Associate 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
Joanne Ekeledo	Project Manager, National Institute for Health and Care Excellence	Present for notes 1 to 25
Ian Watson	Technical Analyst, National Institute for Health and Care Excellence	Present for notes 1 to 15

Aminata Thiam	Technical Adviser, National Institute for Health and Clinical Excellence	Present for notes 1 to 15
Raisa Sidhu	Technical Analyst, National Institute for Health and Care Excellence	Present for notes 16 to 28
Thomas Paling	Technical Adviser, National Institute for Health and Clinical Excellence	Present for notes 16 to 28
Mandy Tonkinson	Administrator National Institute for Health and Clinical Excellence	Present for notes 1 to 22
Rebecca Brandon	Patient Expert	Present for notes 1 to 12
Dr Alex Broomfield	Clinical Expert	Present for notes 1 to 12
Sue Harman	ERG Representative	Present for notes 1 to 12
Dr Derralyn Hughes	Clinical Expert	Present for notes 1 to 12
Onaissa Jamil	Patient Expert	Present for notes 1 to 12
Edmund Jessop	Commissioning Expert	Present for notes 1 to 12 and 16 to 15
Matt Stevenson	ERG Representative	Present for notes 1 to 12
Sophie Thomas	Patient Expert	Present for notes 1 to 12
Rob Hodgson	ERG Representative	Present for notes 16 to 25
Lindsay Claxton	ERG Representative	Present for notes 16 to 25
Matthew Walton	ERG Representative	Present for notes 16 to 25
Dr Ruth Williams	Clinical Expert	Present for notes 16 to 25
Prof Paul Gissen	Clinical Expert	Present for notes 16 to 25
Gail Rich	Patient Expert	Present for notes 16 to 25
Lucy Carroll	Patient Expert	Present for notes 16 to 25
Harriet Lunnemann	Patient Expert	Present for notes 16 to 25

Non-public observers:

Ismahan Abdullah	Administrator National Institute for Health and Clinical Excellence	Present for notes 1 to 22
Helen Barnett	Editor National Institute for Health and Clinical Excellence	Present for all notes
Monique Burger	External observer from VU University	Present for all notes
Ross Dent	Technical Analyst National Institute for Health and Clinical Excellence	Present for all notes
Gavin Kenny	Assistant Project Manager National Institute for Health and Clinical Excellence	Present for all notes
Farah Jamil	External observer, carer for patient expert	Present for 1 to 12
Heidi Livingstone	Public Involvement Adviser National Institute for Health and Clinical Excellence	Present for notes 1 to 23

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 velmanase alfa for treating alpha-mannosidosis and cerliponase alfa for treating neuronal ceroid lipofuscinosis type 2.
2. Apologies were received from Dr Shehla Mohammed and Dr Glenda Sobey.

Any other Business

3. None

Evaluation of velmanase alfa for treating alpha-mannosidosis

Part 1 – Open session

4. The Chair welcomed the invited experts: Rebecca Brandon, Dr Alex Broomfield, Sue Harman, Dr Derralyn Hughes, Onaissa Jamil, Edmund Jessop, Matt Stevenson, Sophie Thomas to the meeting and they introduced themselves to the Committee.
5. The Chair welcomed company representatives from Chiesi Ltd to the meeting.
6. The Chair asked all Committee members to declare any relevant interests
 - 6.1. Professor Ron Akehurst, Dr Ayesha Ali, Sotiris Antoniou, Stuart Davies, Carrie Gardner, Jeremy Manuel, Francis Pang, Linn Phipps, Dr Mark Sheehan, Professor 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 velmanase alfa for treating alpha-mannosidosis.
 - 6.2. Sarah Davis declared a non-personal specific financial interest as part of her team had produced the assessment report for this evaluation, although he had not been directly involved.
 - 6.2.1. It was agreed that this declaration would not prevent Sarah Davis from participating in this section of the meeting.
 - 6.3. Jeremy Manuel declared a personal non-specific financial interest due to his role as co-founder and Director of the Gauchers Association which forms part of the LSD collaborative to which the MPS Society is also a member. The MPS Society support patients with alpha-mannosidosis.
 - 6.3.1. It was agreed that this declaration would not prevent Jeremy Manuel from participating in this section of the meeting.
7. The Chair asked all NICE Staff to declare any relevant interests.
 - 7.1. 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 velmanase alfa for treating alpha-mannosidosis.
8. The Chair asked all other invited guests (assessment group/ERG and invited experts, not including observers) to declare their relevant interests.
 - 8.1. Rebecca Brandon, Sue Harman, Onaissa Jamil, Edmund Jessop and Matt Stevenson 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 velmanase alfa for treating alpha-mannosidosis.
 - 8.2. Dr Alex Broomfield declared a personal non-specific financial interest as he attended an advisory board arranged by Chiesi Ltd.

- 8.2.1. It was agreed that this declaration would not prevent Dr Alex Broomfield from participating in this section of the meeting
- 8.3. Dr Derralynn Hughes declared a personal non-specific financial interest as she attended an advisory board and elicitation panel in relation to velmanase alfa arranged by Chiesi Ltd.
 - 8.3.1. It was agreed that this declaration would not prevent Dr Derralynn Hughes from participating in this section of the meeting
- 8.4. Sophie Thomas declared a personal non-specific financial interest as she attended an advisory board arranged by Chiesi Ltd.
 - 8.4.1. It was agreed that this declaration would not prevent Sophie Thomas from participating in this section of the meeting
9. The Chair introduced the lead team, Dr Ayesha Ali, Linn Phipps and Sarah Davis who gave presentations on the clinical effectiveness and cost effectiveness of velmanase alfa for treating alpha-mannosidosis.
10. The Chair asked the company representatives whether they wished to comment on any matters of factual accuracy.
11. 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.
12. 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

13. Discussion on confidential information continued. This information was supplied by the company.
14. The Committee continued to discuss the clinical and cost effectiveness of velmanase alfa for treating alpha-mannosidosis.
 - 14.1. The committee decision was based on consensus.
15. The Committee instructed the technical team to prepare the Evaluation Consultation Document (ECD) or Final Evaluation Determination (FED) in line with their decisions.

Evaluation of cerliponase alfa for treating neuronal ceroid lipofuscinosis type 2

Part 1 – Open session

16. The Chair welcomed the invited experts: Rob Hodgson, Lindsay Claxton, Matthew Walton, Dr Ruth Williams, Prof Paul Gissen, Gail Rich, Lucy Carroll and Harriet Lunnemann to the meeting and they introduced themselves to the Committee.
17. The Chair welcomed company representatives from BioMarin to the meeting.

18. The Chair asked all Committee members to declare any relevant interests
 - 18.1. Professor Ron Akehurst, Dr Ayesha Ali, Sotiris Antoniou, Stuart Davies, Carrie Gardner, Francis Pang, Linn Phipps and Dr Mark Sheehan 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 cerliponase alfa for treating neuronal ceroid lipofuscinosis type 2.
 - 18.2. Lesley Stewart declared a non-personal specific financial interest as part of her team had produced the assessment report for this evaluation, although he had not been directly involved.
 - 18.2.1. It was agreed that this declaration would not prevent Lesley Stewart from participating in this section of the meeting.
 - 18.3. Jeremy Manuel declared a personal non-specific financial interest due to his role as co-founder and Director of the Gauchers Association which forms part of the LSD collaborative to which the BDFA is also a member.
 - 18.3.1. It was agreed that this declaration would not prevent Jeremy Manuel from participating in this section of the meeting.
19. The Chair asked all NICE Staff to declare any relevant interests.
 - 19.1. 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 cerliponase alfa for treating neuronal ceroid lipofuscinosis type 2.
20. The Chair asked all other invited guests (assessment group/ERG and invited experts, not including observers) to declare their relevant interests.
 - 20.1. Rob Hodgson, Lindsay Claxton, Matthew Walton, Dr Ruth Williams, Gail Rich, Lucy Carroll and Harriet Lunnemann 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 cerliponase alfa for treating neuronal ceroid lipofuscinosis type 2.
 - 20.2. Prof Paul Gissen declared a personal non-specific financial interest as was the UK chief investigator for the BioMarin sponsored clinical trial of cerliponase alpha in CLN2 patients.
 - 20.2.1. It was agreed that this declaration would not prevent Prof Paul Gissen from participating in this section of the meeting
21. 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.

22. The Chair asked the company representatives whether they wished to comment on any matters of factual accuracy.
23. 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.

Part 2a– Closed session

24. The Chair asked clinical and patient experts, ERG and company representatives to remain for part 2a discussions.
25. The Chair thanked the experts and company representatives for their attendance, participation and contribution to the evaluation and they left the meeting.

Part 2b– Closed session

26. Discussion on confidential information continued. This information was supplied by the company.
27. The Committee continued to discuss the clinical and cost effectiveness of cerliponase alfa for treating neuronal ceroid lipofuscinosis type 2.
 - 27.1. The committee decision was based on consensus.
28. The Committee instructed the technical team to prepare the Evaluation Consultation Document (ECD) or Final Evaluation Determination (FED) in line with their decisions.

Date, time and venue of the next meeting

29. Wednesday 23 May 2018 at National Institute for Health and Care Excellence, Level 1A, City Tower, Piccadilly Plaza, Manchester M1 4BT.