

Highly Specialised Technologies Evaluation Committee Meeting

Minutes: Confirmed

Date and Time: Wednesday 17 January 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. Professor Ron Akehurst	Present for all notes
	3. Dr Ayesha Ali	Present for all notes
	4. Sotiris Antoniou	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 Manuel	Present for all notes
	9. Dr Shehla Mohammed	Present for all notes
	10. Francis Pang	Present for all notes
	11. Dr Glenda Sobey	Present for all notes
	12. Professor Lesley Stewart	Present for all notes

In attendance:

Sheela Upadhyaya	Associate Director, National Institute for Health and Care Excellence	Present for all notes
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Joanne Ekeledo	Project Manager, National Institute for Health and Care Excellence	Present for all notes
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Thomas Paling	Technical Lead, National Institute for Health and Care Excellence	Present for all notes
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Raisa Sidhu	Technical Advisor, National Institute for Health and Care Excellence	Present for all notes
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Mandy Tonkinson	Administrator, National Institute for Health and Care Excellence	Present for all notes
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Lucy Carroll	Patient Expert, nominated by Batten	Present for notes 4 to 12
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	Disease Family Association	
Professor Paul Gissen	Clinical Expert, nominated by BioMarin and Batten Disease Family Association	Present for notes 4 to 12
Rob Hodgson	ERG Representative	Present for notes 4 to 12
Edmund Jessop	Commissioning Expert, NHS England	Present for notes 4 to 12
Nick Meader	ERG Representative	Present for notes 4 to 12
Joanne O'Connor	ERG Representative	Present for notes 4 to 12
Gail Rich	Patient Expert, nominated by Batten Disease Family Association	Present for notes 4 to 12
Andrea West	Patient Expert, nominated by Batten Disease Family Association	Present for notes 4 to 12
Dr Ruth Williams	Clinical Expert, nominated by Batten Disease Family Association	Present for notes 4 to 12
Non-public observers:		
Heidi Livingstone	Public Involvement Coordinator, National Institute for Health and Care Excellence	Present for notes 4 to 12
Emma Kent	Data Collection Manager, 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
Karen Samuels	Head of Health Technology Appraisal, Medicines Management and Programme Director, All Wales Therapeutics and Toxicology Centre	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 cerliponase alfa for treating neuronal ceroid lipofuscinosis type 2.
2. The Chair informed the Committee of the non-public observers at this meeting: Heidi Livingstone, Emma Kent, Helen Knight, Karen Samuels and Laura Worthington
3. Apologies were received from Linn Phipps and Mark Sheehan.

Evaluation of cerliponase alfa for treating neuronal ceroid lipofuscinosis type 2

Part 1 – Open session

4. The Chair welcomed the invited experts: Lucy Carroll, Professor Paul Gissen, Rob Hodgson, Edmund Jessop, Nick Meader, Joanne O'Connor, Gail Rich, Andrea West and Dr Ruth Williams to the meeting and they introduced themselves to the Committee.
5. The Chair welcomed company representatives from BioMarin 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, Sarah Davis, Stuart Davies, Carrie Gardner, Dr Shehla Mohammed, Francis Pang and Glenda Sobey 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.
 - 6.2. Professor Lesley Stewart declared a non-personal specific financial interest as part of her team had produced the assessment report for this evaluation, although she had not been directly involved.
 - 9.2.1 It was agreed that this declaration would not prevent Professor Lesley Stewart 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 BDFA is also a member.
 - 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 cerliponase alfa for treating neuronal ceroid lipofuscinosis type 2.
8. The Chair asked all other invited guests (assessment group/ERG and invited experts, including observer Karen Samuels) to declare their relevant interests.
 - 8.1. Lucy Carroll, Rob Hodgson, Edmund Jessop, Nick Meader, Joanne O'Connor, Gail Rich and Andrea West 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.
 - 8.2. Professor Gissen declared a non-personal financial interest as he is he Lead Investigator on clinical trials for CLN2.
 - 8.2.1. It was agreed that this declaration would not prevent Professor Gissen from participating in this section of the meeting
 - 8.3. Dr Ruth Williams declared a personal financial interest as she was involved in the planning and early stages of the clinical trial, and received consultancy payments between 2012 and 2013. She has given presentations at scientific meetings and Symposia arranged and sponsored by BioMarin Pharmaceuticals Inc. She has been involved as a clinical expert in The Centre for Reviews and Dissemination (CRD) and the Centre for Health Economics (CHE) at the University of York's evaluation of the manufacturer's submission as part of this Highly Specialised Technologies (HST) Programme review. Ruth was also Medical Advisor to the BDFA from its inception in 1999 to 2012.
 - 8.3.1. It was agreed that this declaration would not prevent Dr Ruth Williams from participating in this section of the meeting
9. The Chair introduced the lead team, Dr Shehla Mohammed and Professor Ron Akehurst who gave presentations on the clinical effectiveness and cost effectiveness of cerliponase alfa for treating neuronal ceroid lipofuscinosis type 2.
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 cerliponase alfa for treating neuronal ceroid lipofuscinosis type 2.
15. 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

16. Tuesday 20 February 2018 at National Institute for Health and Care Excellence, Level 1A, City Tower, Piccadilly Plaza, Manchester M1 4BT.