

Highly Specialised Technologies Evaluation Committee Meeting

Minutes: Confirmed

Date and Time: Tuesday 17 March 2015, 10.00am to 4.30pm

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. Sotiris Antoniou	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. Francis Pang	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. Sheela Upadhyaya	Present for all notes
	11. Dr Anthony Wierzbicki	Present for all notes

In attendance:

Meindert Boysen	Programme Director, National Institute for Health and Care Excellence	Present for all notes
Josie Godfrey	Associate Director, 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
Ian Watson	Technical Analyst, National Institute for Health and Care Excellence	Present for all notes
Raisa Sidhu	Technical Adviser, National Institute for Health and Clinical Excellence	Present for all notes

Keith Cooper	Southampton Health Technology Assessments Centre (SHTAC)	Present for notes 1 to 15
Neelam Kalita	Southampton Health Technology Assessments Centre (SHTAC)	Present for notes 1 to 15
Emma Loveman	Southampton Health Technology Assessments Centre (SHTAC)	Present for notes 1 to 15
Dr Mohit Jain	BioMarin Inc.	Present for notes 1 to 16
Adrian Quartel	BioMarin Inc.	Present for notes 1 to 16
Dr Fiona Jenkinson	Clinical Expert, nominated by Royal College of Pathologists	Present for notes 1 to 12
Dr Suresh Vijay	Clinical Expert, nominated by British Inherited Metabolic Disease Group	Present for notes 1 to 15
Jibreel Arshad	Patient Expert, nominated by the MPS Society	Present for notes 1 to 15
Anna Eaton	Patient Expert, nominated by the MPS Society	Present for notes 1 to 15
Christine Lavery	Patient Expert, nominated by the MPS Society	Present for notes 1 to 15
Angela Paton	Patient Expert, nominated by the MPS Society	Present for notes 1 to 15
Dr Edmund Jessop	NHS England	Present for notes 1 to 15
Iain Mellis	NHS England	Present for notes 1 to 15

**Non-public observers
(NICE):**

Helen Barnett	Editor	Present for notes 1 to 15
Richard Chivers	Scientific Advice	Present for notes 1 to 15
Heidi Livingstone	Public Involvement Advisor	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 Elosulfase alfa for treating mucopolysaccharidosis type IVA.
2. The Chair informed the Committee of the non-public observers at this meeting: Helen Barnett, Richard Chivers and Heidi Livingstone
3. Apologies were received from Professor Ron Akehurst and Doctor Trevor Cole

Any other Business

4. The Chair reminded Committee Members of the process for evaluation of Highly Specialised Technologies. An update was also provided on the current scope for the Methods Review and the opportunity for Committee Members to provide feedback within this process.

Notes from the last meeting

5. The minutes from the meeting which took place on 09 October 2014 were agreed.

Evaluation of Elosulfase alfa for treating mucopolysaccharidosis type IVA**Part 1 – Open session**

6. The Chair welcomed the invited experts: Dr Fiona Jenkinson, Dr Suresh Vijay, Jibreel Arshad, Anne Eaton, Christine Lavery and Angela Paton to the meeting and they introduced themselves to the Committee.
7. The Chair welcomed representatives from BioMarin Inc. to the meeting.
8. The Chair asked all Committee members to declare any relevant interests
 - 8.1. Sotiris Antoniou, Steve Brennan, Prof Lesley Stewart, Dr Peter Jackson, Linn Phipps and Dr Mark Sheehan 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 Elosulfase alfa for treating mucopolysaccharidosis type IVA .

- 8.2. Dr Anthony Wierzbicki declared a non-personal specific pecuniary interest due to the role of Clinical Investigator for Trust Hospital on studies of lipid lowering compounds for Merck, Pfizer and Amgen
- 8.2.1 It was agreed that this declaration would not prevent Dr Anthony Wierzbicki from participating in this section of the meeting.
- 8.3. Dr Jonathan Howell declared a personal non-specific pecuniary interest due to his wife standing for the 2015 General Election for the National Health Action Party
- 8.3.1 It was agreed that this declaration would not prevent Dr Jonathan Howell from participating in this section of the meeting.
- 8.4. Dr Jonathan Howell declared a non-personal non-specific pecuniary interest due to his role as Consultant in Public Health Post and Representative for NHS England's Rare Disease Advisory Group.
- 8.4.1 It was agreed that this declaration would not prevent Dr Jonathan Howell from participating in this section of the meeting.
- 8.5. Francis Pang declared a non-personal non-specific pecuniary interest due to his role within a healthcare company
- 8.5.1 It was agreed that this declaration would not prevent Francis Pang from participating in this section of the meeting.
- 8.6. Jeremy Manuel declared a non-personal specific pecuniary interest due to his role of Director for the Gauchers Association which is a Member of the LSD Patient Collaborative (of which the MPS Society is also a Member)
- 8.6.1 It was agreed that this declaration would not prevent Jeremy Manuel from participating in this section of the meeting.
- 8.7. Sheela Upadhyaya declared a non-personal specific pecuniary interest due to her role as Specialised Commissioner of Lysosomal storage disorders, extensive work with the MPS Society and member of LSD Expert Advisory Group and Specialised Metabolic CRG
- 8.7.1 It was agreed that this declaration would not prevent Sheela Upadhyaya from participating in this section of the meeting.
- 8.8. Professor Ron Akehurst was not in attendance due to declaring a conflict of interest

Comment [LW1]: Need to state what these conflicts were.....?

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 Elosulfase alfa for treating mucopolysaccharidosis type IVA

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 Elosulfase alfa for treating mucopolysaccharidosis type IVA
11. The Chair introduced the lead team, Dr Jonathan Howell, Prof Lesley Stewart and Linn Phipps who gave presentations on the clinical effectiveness, cost effectiveness and patient perspective of Elosulfase alfa for treating mucopolysaccharidosis type IVA
12. The Committee then discussed the clinical effectiveness, patient perspective and cost effectiveness of Elosulfase alfa for treating mucopolysaccharidosis type IVA on the basis of the evidence before them, and potential equality issues raised in this evaluation. They sought clarification and advice from the experts present. The discussions included:
 - 12.1. The nature and clinical representation of mucopolysaccharidosis type IVA and the impact on quality of life for patients and their family and/or carers
 - 12.2. The clinical effectiveness of Elosulfase Alfa, key clinical trial data and the relevance of the outcome measures used within these
 - 12.3. The cost of Elosulfase Alfa and the budget impact of treating patients in England with mucopolysaccharidosis type IVA
 - 12.4. Economic evidence submitted by the company to estimate the value of elosulfase alfa, and the ERG's critique of this evidence. Discussions included the strengths and limitations of the assumptions and approaches used in the economic model.
 - 12.5. The impact of elosulfase alfa treatment, beyond its direct health benefits, such as social, employment and financial benefits for people with mucopolysaccharidosis type IVA.
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 2 – Closed session

16. Discussion on confidential information continued. This information was supplied by the Company representatives.
17. The Chair then thanked the Company representatives for their attendance, participation and contribution to the evaluation and they left the meeting.
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. Tuesday 9 June 10.00am at National Institute for Health and Care Excellence, Level 1A, City Tower, Piccadilly Plaza, Manchester M1 4BT.