

Highly Specialised Technologies Evaluation Committee Meeting

Minutes: Unconfirmed

Date and Time: Tuesday 21 July 2015, 10.00am - 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. Sotiris Antoniou	Present for all notes
	3. Steve Brennan	Present for all notes
	4. Dr Trevor Cole	Present for all notes
	5. Sarah Davis	Present for all notes
	6. Dr Jonathan Howell	Present for all notes
	7. Jeremy Manuel	Present for all notes
	8. Linn Phipps	Present for all notes
	9. Dr Mark Sheehan	Present for all notes
	10. Prof Lesley Stewart	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
Ahmed Elsada	Technical Analyst, 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
Fiona Pearce	Technical Adviser 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
Keith Cooper	Southampton Health Technology Assessments Centre (SHTAC)	Present for notes 7 to 17
Dr Mohit Jain	BioMarin Inc.	Present for notes 7 to 17
Zlatko Sisic	BioMarin Inc.	Present for notes 7 to 17
Prof Chris Hendriksz	Clinical Specialist	Present for notes 7 to 17
Dr Fiona Jenkinson	Clinical Specialist	Present for notes 7 to 17
Dr Suresh Vijay	Clinical Specialist	Present for notes 7 to 17
Jibreel Arshad	Patient Expert, nominated by the MPS Society	Present for notes 7 to 17
Anna Eaton	Patient Expert, nominated by the MPS Society	Present for notes 7 to 17
Christine Lavery	Patient Expert, nominated by the MPS Society	Present for notes 7 to 17
Angela Paton	Patient Expert, nominated by the MPS Society	Present for notes 7 to 17
Dr Edmund Jessop	NHS England	Present for notes 7 to 17

Non-public observers:

Helen Barnett	Editor	Present for notes 7 to 17
Joost de Folter	Operational Researcher	Present for notes 7 to 19

Eleanor Donegan	Technical Adviser	Present for notes 7 to 17
Linda Landells	Technical Adviser	Present for all notes
Heidi Livingstone	Senior Public Involvement Advisor	Present for all notes
Nwamaka Umeweni	Technical Adviser	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 Elosulfase alfa for treating mucopolysaccharidosis type IVA.
2. The Chair welcomed Sarah Davis to her first meeting as a member of the Evaluation Committee.
3. The Chair informed the Committee of the non-public observers at this meeting: Helen Barnett, Joost de Folter, Eleanor Donegan, Linda Landells, Heidi Livingstone and Nwamaka Umeweni.
4. Apologies were received from Prof Ron Akehurst, Dr Vincent Kirkbride, Francis Pang and Sheela Upadhyaya.

Any other Business

5. The Chair provided an update to Committee Members relating to the following matters:
 - NICE HST team and staffing update
 - Methods/Process Review
 - Topic Selection process
 - Accelerated Access Review
 - Cancer Drugs Fund
 - PPRS - latest position

Notes from the last meeting

6. The minutes from the meeting which took place on 17 March 2015 were agreed.

Evaluation of Elosulfase alfa for treating mucopolysaccharidosis type IVA

Part 1 – Open session

7. The Chair welcomed the invited experts: Prof Chris Hendriksz, 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.

8. The Chair welcomed representatives from BioMarin Inc. to the meeting.
9. The Chair asked all Committee members to declare any relevant interests.
 - 9.1. Sotiris Antoniou, Steve Brennan, Dr Trevor Cole, Sarah Davis, Dr Peter Jackson, Prof Lesley Stewart, 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.
 - 9.2. 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.
 - 9.2.1 It was agreed that this declaration would not prevent Dr Anthony Wierzbicki from participating in this section of the meeting.
 - 9.3. 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.
 - 9.3.1 It was agreed that this declaration would not prevent Dr Jonathan Howell from participating in this section of the meeting.
 - 9.4. Dr Jonathan Howell declared a non-personal non-specific pecuniary interest due his role as Consultant in Public Health Post and Representative for NHS England's Rare Disease Advisory Group.
 - 9.4.1 It was agreed that this declaration would not prevent Dr Jonathan Howell from participating in this section of the meeting.
 - 9.5. 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).
 - 9.5.1 It was agreed that this declaration would not prevent Jeremy Manuel from participating in this section of the meeting.
10. Professor Ron Akehurst was not in attendance due to declaring a conflict of interest.
11. The Chair asked all NICE Staff to declare any relevant interests.
 - 11.1. 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.

12. The Chair asked all other invited guests, ERG and invited experts, not including observers) to declare their relevant interests.
 - 12.1. 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.
 - 12.2. Prof Chris Hendriksz declared a non-personal specific pecuniary interest as he been employed by BioMarin and other companies/patient organisations which are linked to mucopolysaccharidosis type IVA.
 - It was agreed that this declaration would not prevent Prof Chris Hendriksz from participating in this section of the meeting.
13. The Chair summarised the key issues discussed by Committee during the first meeting in March 2015, which led to the preliminary recommendation in the Evaluation Consultation Document. He then summarised the key themes arising from the consultation responses to the Evaluation Consultation Document (ECD) received from consultees, commentators and through the NICE website.
14. The Committee then discussed the responses from consultation and considered the clinical effectiveness, patient perspective, value for money and budget impact of elosulfase alfa for treating mucopolysaccharidosis type IVA on the basis of the evidence before them in this evaluation. They sought clarification and advice from the experts present. The discussions included:
 - 14.1. The nature and clinical representation of mucopolysaccharidosis type IVA and the impact on quality of life for patients and their family and/or carers with an emphasis on the wider impact of the disease beyond the effects on the skeletal system
 - 14.2. Additional analyses in response to consultation from the company, including:
 - The cost of elosulfase alfa (in the context of developing and manufacturing)
 - The benefits that the treatment provides to people with the condition
 - Further economic analyses
 - Budget impact for the NHS and Personal Social Services with particular reference to administering elosulfase alfa within people's homes
 - Further details of the criteria for starting and stopping treatment
15. The Chair asked the company representatives whether they wished to comment on any matters of factual accuracy.
16. 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.

17. The Chair then invited the experts and company representatives to stay for the first part of the closed session if they wished to make any final statements in private.

Part 2 – Closed session

18. The company representatives and experts made closing statements and answered the Committee's questions regarding confidential information that could not be discussed during the public part of the meeting.
19. The Chair then thanked the experts and company representatives for their attendance, participation and contribution to the evaluation and they left the meeting.
20. 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

21. Thursday 17 September 2015 at National Institute for Health and Care Excellence, Level 1A, City Tower, Piccadilly Plaza, Manchester M1 4BT.