

## Highly Specialised Technologies Evaluation Committee Meeting

**Minutes:** Confirmed

**Date and Time:** Tuesday 22 November 2016, 10am to 4:30pm

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

<b>Present:</b>	1. Peter Jackson, Chair 2. Sotiris Antoniou 3. Sarah Davis 4. Carrie Gardner 5. Jonathan Howell 6. Francis Pang 7. Linn Phipps 8. Mark Sheehan 9. Glenda Sobey 10. Lesley Stewart	Present for all notes Present for 18 to 29 Present for all notes Present for all notes Present for all notes Present for all notes
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### In attendance:

Meindert Boysen	Programme Director, National Institute for Health and Care Excellence	Present for all notes
Kimberley Carter	Technical Analyst, National Institute for Health and Care Excellence	Present for 1 to 17
Jenna Dilkes	Project Manager, National Institute for Health and Care Excellence	Present for all notes
Boglarka Mikudina	Technical Lead, National Institute for Health and Care Excellence	Present for 30 to 42
Thomas Strong	Technical Lead, National Institute for Health and Care Excellence	Present for 18 to 29
Sheela Upadhyaya	Associate Director, National Institute for	Present for all notes

	Health and Care Excellence	
Ian Watson	Technical Adviser, National Institute for Health and Clinical Excellence	Present for all notes
Patrick Deegan	Clinical Expert, Royal College of Pathologists	Present for 30 to 38
Edmund Jessop	NHS England	Present for all notes
Simon Jones	Clinical Expert, Wilink Unit, Alexion and the MPS Society	Present for 30 to 38
Stuart Lancaster	Patient Expert, MPS Society	Present for 30 to 38
Zulf Mughal	Clinical Expert, Royal Manchester Children's Hospital	Present for 18 to 26
Sophie Thomas	Patient Expert, MPS Society	Present for 30 to 38
Michael Whitworth	NHS England	Present for all notes
Jeff Castelli	Senior Vice President, Amicus	Present for 1 to 14
Emma Harvey	Senior Medical Director, Alexion	Present for 18 to 26 and 30 to 38
Alasdair MacCulloch	Regional Market Access Director, Amicus	Present for 1 to 14
Sarah Pitluck	Executive Director, Alexion	Present for 18 to 26 and 30 to 38
Heidi Wagner	Senior Vice President, Alexion	Present for 18 to 26 and 30 to 38
Sara Trafford Jones	VP and General Manager, Alexion	Present for 18 to 26 and 30 to 38

**Non-public observers:**

Helen Barnett	Editor National Institute for Health and Care Excellence	Present for all 18 to 42
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Ann Greenwood	Editor National Institute for Health and Care Excellence	Present for 1 to 17
Heidi Livingstone	Public Involvement Programme National Institute for Health and Care Excellence	Present for 1 to 17 and 30 to 42
Joost de Folter	Scientific Advice NICE	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 evaluations of **Migalastat for treating Fabry disease**, **Asfotase alfa for treating paediatric-onset hypophosphatasia** and **Sebelipase alfa for treating lysosomal acid lipase deficiency**.
2. The Chair welcomed Carrie Gardner and Dr Glenda Sobey to their 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, Heidi Livingstone and Joost de Folter.
4. Apologies were received from Ron Akehurst, Vincent Kirkbridge, Jeremy Manuel and Shehla Mohammed.

### Any other Business

5. None

### Evaluation of Migalastat for treating Fabry disease

#### Part 1 – Open session

6. The Chair welcomed the invited experts: Edmund Jessop and Michael Whitworth to the meeting and they introduced themselves to the Committee.
7. The Chair welcomed company representatives from Amicus to the meeting.
8. The Chair asked all Committee members to declare any relevant interests
  - 8.1. Sotiris Antoniou, Sarah Davis, Carrie Gardner, Peter Jackson, Jonathan Howell, Mark Sheehan, Glenda Sobey and 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 Migalastat for treating Fabry disease.

8.2. Linn Phipps declared a personal non-specific financial 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. Francis Pang was absent due to conflicts. Francis Pang declared a personal non-specific financial interest due to his relationship to the company of the topic under evaluation.  
8.3.1. It was agreed that this declaration would prevent Francis Pang from participating in the meeting.

9. The Chair asked all NICE Staff to declare any relevant interests.

9.1. 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 Migalastat for treating Fabry disease.

10. The Chair asked all other invited guests (ERG and invited experts, not including observers) to declare their relevant interests.

10.1. 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 Migalastat for treating Fabry disease.

11. 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.

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

13. 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.

14. 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**

15. Discussion on confidential information continued. This information was supplied by the company.

16. The Committee continued to discuss the clinical and cost effectiveness of Migalastat for treating Fabry disease.

17. 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 Asfotase alfa for treating paediatric-onset hypophosphatasia**

### **Part 1 – Open session**

18. The Chair welcomed the invited experts: Edmund Jessop, Zulf Mughal and Michael Whitworth to the meeting and they introduced themselves to the Committee.
19. The Chair welcomed company representatives from Alexion to the meeting.
20. The Chair asked all Committee members to declare any relevant interests
  - 20.1. Sotiris Antoniou, Sarah Davis, Carrie Gardner, Peter Jackson, Jonathan Howell, Mark Sheehan, Glenda Sobey and 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 Asfotase alfa for treating paediatric-onset hypophosphatasia.
  - 20.2. Linn Phipps declared a personal non-specific financial interest due to her role as Patient & Public Voice Representative on NHS England's CPAG (Clinical Priorities Advisory Group).
    - 20.2.1. It was agreed that this declaration would not prevent Linn Phipps from participating in the meeting.
  - 20.3. Francis Pang declared a personal non-specific financial interest due to his role within the healthcare industry.
    - 20.3.1. It was agreed that this declaration would not prevent Francis Pang from participating in the meeting.
21. The Chair asked all NICE Staff to declare any relevant interests.
  - 21.1. 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 Asfotase alfa for treating paediatric-onset hypophosphatasia.
22. The Chair asked all other invited guests (ERG and invited experts, not including observers) to declare their relevant interests.
  - 22.1. 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 Asfotase alfa for treating paediatric-onset hypophosphatasia.

23. 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.
24. The Chair asked the company representatives whether they wished to comment on any matters of factual accuracy.
25. 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.
26. 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**

27. Discussion on confidential information continued. This information was supplied by the company.
28. The Committee continued to discuss the clinical and cost effectiveness of Asfotase alfa for treating paediatric-onset hypophosphatasia.
29. 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 Sebelipase alfa for treating lysosomal acid lipase deficiency**

### **Part 1 – Open session**

30. The Chair welcomed the invited experts: Patrick Deegan, Edmund Jessop, Simon Jones, Stuart Lancaster, Sophie Thomas, Michael Whitworth to the meeting and they introduced themselves to the Committee.
31. The Chair welcomed company representatives from Alexion to the meeting.
32. The Chair asked all Committee members to declare any relevant interests
  - 32.1. Sotiris Antoniou, Sarah Davis, Carrie Gardner, Peter Jackson, Jonathan Howell, Mark Sheehan, Glenda Sobey and 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 Sebelipase alfa for treating lysosomal acid lipase deficiency.
  - 32.2. Linn Phipps declared a personal non-specific financial interest due to her role as Patient & Public Voice Representative on NHS England's CPAG (Clinical Priorities Advisory Group).
    - 32.2.1. It was agreed that this declaration would not prevent Linn Phipps from participating in the meeting.

33. The Chair asked all NICE Staff to declare any relevant interests.
  - 33.1. 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 Sebelipase alfa for treating lysosomal acid lipase deficiency.
34. The Chair asked all other invited guests (assessment group/ERG and invited experts, not including observers) to declare their relevant interests.
  - 34.1. 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 Sebelipase alfa for treating lysosomal acid lipase deficiency.
35. 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.
36. The Chair asked the company representatives whether they wished to comment on any matters of factual accuracy.
37. 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.
38. 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**

39. Discussion on confidential information continued. This information was supplied by the company.
40. The Committee continued to discuss the clinical and cost effectiveness of Sebelipase alfa for treating lysosomal acid lipase deficiency.
41. 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**

42. Thursday 19 January 2017 from 10am to 5pm at National Institute for Health and Care Excellence, Level 1A, City Tower, Piccadilly Plaza, Manchester M1 4BT.