

## Highly Specialised Technologies Evaluation Committee Meeting

**Minutes:** Confirmed

**Date and Time:** Thursday 28 September 2017

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

<b>Present:</b>	1. Dr Peter Jackson	Present for all notes
	2. Prof Ron Akehurst	Present for all notes
	3. Sotiris Antoniou	Present for all notes
	4. Sarah Davis	Present for all notes
	5. Stuart Davies	Present for all notes
	6. Dr Vincent Kirkbride	Present for all notes
	7. Jeremy Manual	Present for all notes
	8. Dr Shehla Mohammed	Present for all notes
	9. Francis Pang	Present for all notes
	10. Linn Phipps	Present for all notes
	11. Dr Glenda Sobey	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
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 all notes
Ian Watson	Technical Analyst, National Institute for Health and Care Excellence	Present for all notes
Thomas Strong	Technical Adviser, National Institute for Health and Clinical Excellence	Present for all notes

Mandy Tonkinson	Administrator National Institute for Health and Clinical Excellence	Present for all notes
Professor Alessandro Aiuti	Clinical Expert (nominated by GSK)	Present for notes 1 to 13
Dr Claire Booth	Clinical Expert (nominated by GOSH)	Present for notes 1 to 13
Ed Cox	ERG Representative	Present for notes 1 to 13
Susan Griffin	ERG Representative	Present for notes 1 to 13
Edmund Jessop	NHS Commissioning Expert	Present for notes 1 to 13
Fiona Marley	NHS Commissioning Expert	Present for notes 1 to 13
Nick Meader	ERG Representative	Present for notes 1 to 13
Claire Reid	Patient Expert (nominated by PID UK)	Present for notes 1 to 13
Susan Walsh	Patient Expert (nominated by PID UK)	Present for notes 1 to 13

**Non-public observers:**

Helen Barnett	Editor, National Institute for Health and Clinical Excellence	Present for all notes
Heidi Livingstone	Public Involvement Advisor, National Institute for Health and Clinical Excellence	Present for all notes
Nick Crabb	Programme Director, Scientific Affairs, National Institute for Health and Clinical Excellence	Present for all notes
Pilar Pinilla-Dominguez	Technical Adviser – National Institute for Health and Clinical Excellence	Present for all notes
Sophie Cooper	Technical Adviser – National Institute for Health and Clinical	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 strimvelis for treating severe combined immunodeficiency caused by adenosine deaminase deficiency
2. The Chair informed the Committee of the non-public observers at this meeting: Helen Barnett, Heidi Livingstone, Nick Crabb, Pilar Pinilla-Dominguez, Sophie Cooper and Emily South
3. Apologies were received from Dr Ayesha Ali, Carrie Gardner and Dr Mark Sheehan.

### Any other Business

4. None

### Evaluation of Strimvelis for treating severe combined immunodeficiency caused by adenosine deaminase deficiency

#### Part 1 – Open session

5. The Chair welcomed the invited experts: Professor Alessandro Aiuti, Dr Claire Booth, Ed Cox, Susan Griffin, Edmund Jessop, Fiona Marley, Nick Meader, Claire Reid and Susan Walsh to the meeting and they introduced themselves to the Committee.
6. The Chair welcomed company representatives from GlaxoSmithKline to the meeting.
7. The Chair asked all Committee members to declare any relevant interests
  - 7.1. Prof Ron Akehurst, Sotiris Antoniou, Sarah Davis, Stuart Davies, Dr Vincent Kirkbride, Jeremy Manual, Dr Shehla Mohammed, Francis Pang, Linn Phipps, Dr Glenda Sobey and Prof 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 strimvelis for treating severe combined immunodeficiency caused by adenosine deaminase deficiency.
8. The Chair asked all NICE Staff to declare any relevant interests.
  - 8.1. 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 strimvelis for treating severe combined immunodeficiency caused by adenosine deaminase deficiency.

9. The Chair asked all other invited guests (assessment group/ERG and invited experts, not including observers) to declare their relevant interests.
  - 9.1. Ed Cox, Susan Griffin, Edmund Jessop, Fiona Marley, Nick Meader, and Claire Reid 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 strimvelis for treating severe combined immunodeficiency caused by adenosine deaminase deficiency
  - 9.2. Prof Alessandro Aiuti declared a personal financial interest as he received consultancy fees from GlaxoSmithKline, a non-personal financial interest as the organisation that he works for receives milestone payments and royalties from GlaxoSmithKline and a personal non-financial interest as he has published scientific papers on ADA-SCID.
    - 9.2.1. It was agreed that this declaration would not prevent Prof Alessandro Aiuti from participating in this section of the meeting
  - 9.3. Dr Claire Booth declared a non-personal specific financial interest as she is the Principal Investigator of two Phase I/II lentiviral clinical trials for ADA-SCID and Co-Investigator on 4 other open trials and 2 in set-up.
    - 9.3.1. It was agreed that this declaration would not prevent Dr Claire Booth from participating in this section of the meeting.
  - 9.4. Susan Walsh declared a non-personal financial interest as the organisation she is Director of (PID UK) has received unrestricted funding from Orchard Therapeutics – a company with an interest in commercial gene therapy for this condition and other rare primary immunodeficiencies.
    - 9.4.1. It was agreed that this declaration would not prevent Susan Walsh from participating in this section of the meeting.
10. The Chair introduced the lead team, Dr Vincent Kirkbride, Jeremy Manuel and Sarah Davis who gave presentations on the clinical effectiveness and cost effectiveness of strimvelis for treating severe combined immunodeficiency caused by adenosine deaminase deficiency
11. The Chair asked the company representatives whether they wished to comment on any matters of factual accuracy.
12. 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.

13. 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**

14. Discussion on confidential information continued. This information was supplied by the company.
15. The Committee continued to discuss the clinical and cost effectiveness of strimvelis for treating severe combined immunodeficiency caused by adenosine deaminase deficiency
16. 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**

17. Thursday 17 October 2017 at National Institute for Health and Care Excellence, Level 1A, City Tower, Piccadilly Plaza, Manchester M1 4BT.