# Highly Specialised Technologies Evaluation Committee Meeting

Minutes: Confirmed

Date and Time: Thursday 25 July 2019

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.Sotiris Antoniou Present for all notes

3.Paul Arundel Present for all notes

4.Sarah Davis Present for all notes

5.Stuart Davies Present for all notes

6.Carrie Gardner Present for all notes

7.Jeremy Manuel Present for all notes

8.Dr Shehla Mohammed Present for all notes

9.Linn Phipps Present for all notes

10.Dr Mark Sheehan Present for all notes

11.Professor Lesley Stewart Present for all notes

## **In attendance:**

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

Rashida Akhtar, HST Administrator

National Institute for Health and Care Excellence Present for all notes

Louise Jafferally, Project Manager

National Institute for Health and Care Excellence Present for all notes

Yelan Guo, Technical Adviser

National Institute for Health and Care Excellence Present for all notes

Lorna Dunning, Technical Lead,

National Institute for Health and Clinical Excellence Present for all notes

Mandy Tonkinson, Public Involvement Adviser

National Institute for Health and Clinical Excellence Present for all notes

Ayesha Ali, NHSE commissioning expert Present for notes 1-8

John Spoors, NHSE Expert Present for notes 1-8

Tina Houlihan, Patient expert Present for notes 1-8

Robert Johnson, Patient Expert Present for notes 1-8

Robert Maclaren, Clinical expert Present for notes 1-8

Andrew Webster, Clinical Expert Present for notes 1-8

Caroline Farmer, Evidence Review Group (PenTAG) Present for notes 1-8

David Packman, Evidence Review Group (PenTAG) Present for notes 1-8

G.J. Melendez Torres, Evidence Review Group (PenTAG) Present for notes 1-8

Sue Lacey, Novartis Present for notes 1-8

Raisa Sidhu. Novartis Present for notes 1-8

# 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 Voretigene neparvovec for treating inherited retinal dystrophies caused by RPE65 gene mutations and Cerliponase alfa for treating neuronal ceroid lipofuscinosis type 2 (Part 2 private discussion).
2. The Chair informed the Committee of the non-public observers at this meeting: Jan Jones, Ailsa Brown, Julia McCombie, Alison Strath, Karen Facey, Amanda Whittles, Trenholme Junghans, Ken Bound, and Jeff Round.
3. Apologies were received from Professor Ron Akehurst, Francis Pang, Matt Smith and Dr Glenda Sobey.

Any other Business

1. None

# Evaluation of Voretigene neparvovec for treating inherited retinal dystrophies caused by RPE65 gene mutations.

## **Part 1 – Open session**

1. The Chair welcomed the invited experts: Ayesha Ali, John Spoors, Tina Houlihan, Robert Johnson, Robert Maclaren, Andrew Webster to the meeting and they introduced themselves to the Committee.
2. The Chair welcomed company representatives from Novartis to the meeting.
3. The Chair asked all Committee members to declare any relevant interests

All present Committee members 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 Voretigene neparvovec for treating inherited retinal dystrophies caused by RPE65 gene mutations.

1. The Chair asked all NICE Staff to declare any relevant interests.
   1. All NICE Staff 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 Voretigene neparvovec for treating inherited retinal dystrophies caused by RPE65 gene mutations.
2. The Chair asked all other invited guests (assessment group/ERG and invited experts, not including observers) to declare their relevant interests.
   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 Voretigene neparvovec for treating inherited retinal dystrophies caused by RPE65 gene mutations.
3. The Chair introduced the lead team, Carrie Gardner, Dr Shehla Mohammed and Linn Phipps who gave presentations on the clinical effectiveness and cost effectiveness of Voretigene neparvovec for treating inherited retinal dystrophies caused by RPE65 gene mutations.
4. The Chair asked the company representatives whether they wished to comment on any matters of factual accuracy.
5. 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.
6. 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**

1. Discussion on confidential information continued. This information was supplied by the company. Clinical and patient experts, ERG and company representatives excused.
2. The Committee continued to discuss the clinical and cost effectiveness of Voretigene neparvovec for treating inherited retinal dystrophies caused by RPE65 gene mutations.
3. The committee decision was based on consensus.
4. The Committee instructed the technical team to prepare the Final Evaluation Determination (FED)] in line with their decisions.

# Part 2 private discussion - Evaluation of Cerliponase alfa for treating neuronal ceroid lipofuscinosis type 2 [ID943].

1. Discussion on confidential information. This information was supplied by the company.
2. The Committee agreed to defer a decision on the content of the guidance section of the Final Evaluation Determination (FED) until 29 August 2019

# Date, time and venue of the next meeting

1. Thursday 29 August 2019, 10am to 5pm at National Institute for Health and Care Excellence, Level 1A, City Tower, Piccadilly Plaza, Manchester M1 4BT.