Human Fertilisation and Embryology Authority

Minutes of the Statutory Approvals Committee

Meeting held at Finsbury Tower, 103-105 Bunhill Row, London, EC1Y 8HF on
26 February 2015

Minutes – Item 4

Centre 0102 (Guys Hospital) – PGD application for mucopolysaccharidosis IVA OMIM #253000

Members of the Committee:	David Archard (Chair, lay) Jane Dibblin (lay) Sue Price (professional) Tony Rutherford (professional)
Legal Adviser:	Philip Grey, Mills and Reeve
Specialist Attending:	Dr Anne Lampe
Members of the Executive:	Sam Hartley – Head of Governance and Licensing Trent Fisher – Secretary

Declarations of Interest: members of the committee declared that they had no conflicts of interest in relation to this item.

The following papers were considered by the committee:

- Executive Summary
- PGD application form
- Redacted peer review
- Genetic alliance opinion

The committee also had before it:

- HFEA Protocol for the Conduct of Licence Committee Meetings and Hearings
- 8th edition of the HFEA Code of Practice
- Human Fertilisation and Embryology Act 1990 (as amended)
- Decision trees for granting and renewing licences and considering requests to vary a licence (including the PGD decision tree); and
- Guidance for members of Authority and Committees on the handling of conflicts of interest approved by the Authority on 21 January 2009.

- Guidance on periods for which new or renewed licences should be granted
- Standing Orders and Instrument of Delegation
- Indicative Sanctions Guidance
- HFEA Directions 0000 – 0012
- Guide to Licensing
- Compliance and Enforcement Policy
- Policy on Publication of Authority and Committee Papers

Discussion

1. The committee had regard to its Decision Tree. The committee noted that the centre is licensed to carry out PGD. The committee was also satisfied that the centre has experience of carrying out PGD and that generic patient information about its PGD programme and associated consent forms had previously been received by the HFEA.

2. The committee noted that the condition being applied for is not on the approved PGD conditions list.

3. The committee noted that the proposed purpose of testing the embryos was as set out in paragraph 1ZA(1)(b) of schedule 2 of the Act, i.e. 'where there is a particular risk that the embryo may have any gene, chromosome or mitochondrion abnormality, establishing whether it has that abnormality or any other gene, chromosome or mitochondrion abnormality'.

4. The committee noted that mucopolysaccharidosis IVA OMIM #253000 is inherited in an autosomal recessive pattern and there is a 1 in 4 chance of an embryo being affected with the condition where both parents are carriers.

5. The committee noted that individuals affected by this condition suffer from progressive bone and joint problems which limit mobility leading to pain and arthritis. Compression of the spinal cord is a common complication that may result in neurological impairment. Involvement of other organ systems leads to significant morbidity including respiratory compromise, obstructive sleep apnea and valvular heart disease.

6. The committee noted that other symptoms that may develop are hip problems causing pain and stiffness, kyphoscoliosis, knock knee and pectus carinatum. Hearing impairment, visual impairment from corneal clouding, dental abnormalities, and hepatomegaly are also associated with this condition.

7. The committee noted that the condition is fully penetrant and is extremely variable making it hard to predict the symptoms an individual may develop. The symptoms can be present from infancy and early childhood.

8. The committee noted that recently an enzyme replacement therapy has been licenced to treat mucopolysaccharidosis IVA. The initial results have been promising however it is not clear what the effects the treatment will have in the long term. Other treatment options available are only those to manage some of the symptoms that arise from the condition.

9. The committee noted that the application is consistent with the Peer Review.

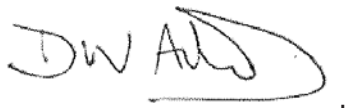
10. The committee welcomed the advice of its advisor, Dr Anne Lampe. Dr Lampe pointed out that the description contained at paragraph 4.2 of the Executive Summary was inaccurate with the reference to 'Symptoms usually present in a mild form at birth…' as symptoms of the condition do not present until after birth. Dr Lampe confirmed that, subject to the matters referred to by this correction, the condition is as described in the papers and added that due to the skeletal, respiratory and neurological issues this is a severely debilitating condition.

11. The committee considered that the condition is serious due to the complete penetrance, the extreme number and variability of symptoms, the neurological issues that can arise and the significant morbidity.

12. The committee had regard to its explanatory note and noted that on the basis of the information presented, given the condition's worst symptoms, it was satisfied that there is a significant risk that a person with the abnormality will have or develop a serious physical or mental disability, a serious illness or any other serious medical condition. The committee was therefore satisfied that the condition meets the criteria for testing under paragraph 1ZA(1)(b) and (2) of Schedule 2 to the Act.

13. The committee agreed to authorise the testing of embryos for mucopolysaccharidosis IVA OMIM #253000.

Signed: Date: 12 March 2015



David Archard (Chair)