HFEA Licence Committee Meeting

14 June 2012

Finsbury Tower, 103-105 Bunhill Row, London, EC1Y 8HF

Minutes – Item 3

Centre 0070 (The Bridge) – PGD for Progressive Familial Intraheptic ~~Chloestasis~~ Cholestasis Type 1 (PFIC1) (OMIM #211600)

Members of the Committee:	Committee Secretary:
David Archard (lay) Chair Mair Crouch (lay) Sue Price (professional) Debbie Barber (professional) Jane Dibblin (lay)	Lauren Crawford Legal Adviser: Juliet Oliver, Field Fisher Waterhouse

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

- Cover sheet
- 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
- HFEA Pre-Implantation Diagnostic Testing ("PGD") Explanatory Note For Licence Committee

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 Centre's proposed purpose of testing the embryos was as set out in paragraph 1ZA(1)(b) of schedule 2 of the Act, ie. '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'.

3. The Committee noted Progressive Familial Intraheptic ~~Chloestasis~~Cholestasis Type 1 (PFIC1) (OMIM #211600) is a disorder that is inherited in an autosomal recessive manner. There is a 1 in 4 chance of an embryo being affected by this condition where a parent is affected.

4. The Committee noted Progressive Familial Intraheptic ~~Chloestasis~~Cholestasis Type 1 (PFIC1) is a childhood disorder that disrupts bile formation preventing the flow and producing severe liver damage. The condition usually develops in the first few months of life. Life expectancy is short, death usually occurring in the first few months of life, or in the first 1-5 years with clinical intervention.

5. The Committee noted PFIC1 is characterized by the following problems: poor weight gain (due to a lack of bile needed to digest and absorb fat) and poor growth; poor feeding, nausea and vomiting; difficulty absorbing fats and fat-soluble vitamins (D, E, A, K); jaundice; failure to thrive; abnormal enlargement of the liver and spleen; severe itching caused by the build up of bile salt in the body (pruritus) and cirrhosis leading to liver failure. Treatments offered include ursodeoxycholic acid (UDCA) therapy to prevent liver damage. In some PFIC1 patients, biliary diversion can also relieve pruritus and slow disease progression. However, as described above, the condition often leads to cirrhosis of the liver with liver transplant being the only clinical option available to prevent early death.

6. The Committee noted that the condition is serious because Progressive Familial Intrahepatic Cholestasis presents in early infancy. The disorder is fully penetrant. In PFIC1, the infant presents with recurrent jaundice in the first few months of life. This is accompanied by severe itching, poor feeding, chronic diarrhoea, failure to thrive. These children go on to develop portal hypertension, cirrhosis and liver failure. The underlying absence of bile ducts leads to liver fibrosis and cirrhosis and the only definitive treatment is liver transplant. It has been reported that the cirrhosis can develop even in the transplanted organ.

7. The Committee noted that the application is supported by the Peer Reviewer as well as by Genetic Alliance UK.

8. The Committee 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. Accordingly, it was appropriate to grant the application under paragraph 1ZA(1)(b) of Schedule 2 to the Act.

9. The Committee noted that the application is supported by Genetic Alliance UK.

10. The Committee noted that while they may grant approval, the centre providing PGD for the condition must also ensure that each individual case treated fulfills the severity criteria of Schedule 2 paragraph 1ZA of the HFE Act 1990 (as amended).

11. The Committee agreed to authorise the testing of embryos for Progressive Familial Intrahepatic Cholestasis Type 1 (PFIC1) (OMIM #211600). The Committee confirmed that this condition will be added to the published list of conditions for which PGD may be carried out.

Signed: Date: 27/06/2012

David Archard (Chair)