Statutory Approvals Committee – minutes

Centre 0044 (The Centre for Reproductive and Genetic Health) Pre-implantation Genetic Diagnosis (PGD) application for Charcot-Marie-Tooth disease, axonal, type 2P (CMT2P), OMIM #614436

Thursday, 25 October 2018

Church House Westminster, Dean's Yard, Westminster SW1P 3NZ

Committee members	Margaret Gilmore (Chair) Bobbie Farsides (Deputy Chair) Anne Lampe Anthony Rutherford Ruth Wilde Emma Cave (New Member Induction - Observer)	
Members of the Executive	Dee Knoyle Bernice Ash Catherine Burwood Paula Robinson	Committee Secretary Committee Secretary (Observer) Senior Governance Manager (Observer) Head of Planning and Governance (Observer)
Specialist Adviser	Dr Mary Porteous	
Legal Adviser	Gerard Hanratty	Browne Jacobson LLP
Observers		

Declarations of interest

- Members of the committee declared that they had no conflicts of interest in relation to this item.

The committee had before it:

- 8th edition of the HFEA Code of Practice
- Standard licensing and approvals pack for committee members

The following papers were considered by the committee:

- Executive summary
- PGD application form
- Redacted peer review
- Genetic Alliance UK statement
- 2011-03-31: Licence Committee minutes - PGD for Hereditary Motor and Sensory Neuropathy 1A (Charcot Marie Tooth type 1A) - Refused
- 2012-01-26 Licence Committee Minutes - PGD for Charcot Marie Tooth Disease Type 2
- 2012-07-22 Licence Committee minutes - PGD for Charcot Marie Tooth Disease Type 1A
- 2014-01-30 Statutory Approvals Committee Minutes - PGD for Distal hereditary Motor Neuropathy type IIB
- 2018-05-24 Statutory Approvals Committee Minutes - PGD for Charcot Marie Tooth type 1B (CMT1B)

1. Consideration of application

1.1. The committee welcomed the advice of its Specialist Adviser, Dr Mary Porteous who confirmed that the condition was as described in the papers.

1.2. The committee noted that the description in the application for PGD for Charcot-Marie-Tooth disease, axonal, type 2P (CMT2P), OMIM #614436 is consistent with the peer review.

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

1.4. The committee noted that the Genetic Alliance opinion provided a patient perspective and supported the application.

1.5. 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.

1.6. 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'.

1.7. The committee noted that the condition is inherited in an autosomal dominant pattern which means there is a 50% chance of an embryo being affected with the condition in each pregnancy, if either parent has a relevant mutation.

1.8. The committee noted that penetrance of the condition is very high, with the majority of patients showing clinical symptoms by the time they are adults, but an exact measure of penetrance has not been provided in the application. The condition causes damage to peripheral sensory and motor nerves, although motor deficits are more prominent than sensory deficits. Typically, lower extremities are affected earlier and more severely than upper extremities but damage to the nerves becomes more distal in upper extremities as the disease progresses. Symptoms can become apparent anytime from early childhood through to late adulthood but more typically develop in teenagers. Symptoms vary in severity, even within families, ranging from extremely mild disease that goes unrecognised by the affected individual and physician, to considerable weakness and disability requiring crutches / wheelchair. CMT2P is said to be similar in symptoms and impact to CMT2A (OMIM #609260) and to Distal Hereditary Motor Neuropathy, type IIB OMIM 608634, both of which are licensed for PGD.

1.9. The committee noted that treatment is not curative but aimed at managing symptoms. Affected individuals should be managed by a multidisciplinary team including a neurologist, physiatrist, orthopaedic surgeon, and physical and occupational therapists.

1.10. The committee noted the inspectorate's request to consider whether CMT2P, OMIM #614436 should be approved for inclusion on the list of conditions approved for PGD. The committee agreed to consider the application on this basis.

2. Decision

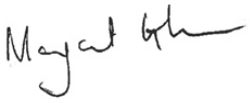
2.1. The committee considered that, in the worst case scenario CMT2P, OMIM #614436 is a serious progressive disabling condition which causes weakness, mainly in the lower limbs, and balance problems affecting mobility. The age of onset is early childhood and individuals experience musculoskeletal and neuropathic pain and require the use of crutches or a wheelchair for mobility. The quality of life for affected individuals is severely impacted and there is no curative treatment.

2.2. The committee had regard to its explanatory note and confirmed that, on the basis of the information presented, it was satisfied that there is a particular risk that an embryo may have the abnormality in question and that there is a significant risk, given the condition's worst symptoms, 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 Charcot-Marie-Tooth disease, axonal, type 2P (CMT2P), OMIM #614436 meets the criteria for testing under paragraph 1ZA(1)(b) and (2) of Schedule 2 of the Act and agreed to authorise testing.

3. Chairs signature

3.1. I confirm this is a true and accurate record of the meeting.

Signature



Name

Margaret Gilmore

Date

27 November 2018