# Statutory Approvals Committee – minutes

## Centre 0102 (Guys Hospital)

## Pre-implantation Genetic Diagnosis (PGD) application for Wolfram-Like Syndrome (WLS), OMIM #614296

Thursday, 26 November 2020

HFEA, 10 Spring Gardens, London, SW1A 2BU via Teams Meeting

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| Committee members | Margaret Gilmore (Chair)<br>Emma Cave<br>Anne Lampe<br>Ruth Wilde | |
| Members of the Executive | Moya Berry | Committee Officer |
| Specialist Adviser | Professor Mary Porteous | |
| Legal Adviser | Eve Piffaretti | Blake Morgan – LLP |

## 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:

- 9th 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

## 1. Consideration of application

**1.1.** The committee welcomed the advice of its specialist adviser, Professor Mary Porteous, who confirmed that the condition was as described in the papers.

**1.2.** The committee noted that the description in the PGD application for Wolfram-like syndrome (WLS), OMIM #614296, is consistent with the peer review.

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

1.4. The committee noted that a Genetic Alliance UK statement had not been provided for this 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 Wolfram-like syndrome (WLS), OMIM #614296, is inherited in an autosomal dominant manner, which means there is a 50% chance of an embryo being affected by the condition in each pregnancy if either parent has a relevant mutation.

1.8. The committee noted that the penetrance of the condition is unknown.

1.9. Wolfram-like syndrome (WLS), OMIM #614296, is a progressive neurodegenerative disorder characterised by visual and hearing impairment, and diabetes. The condition can develop in childhood and may be severe, affecting speech and communication, learning and social interaction. The co-occurrence of visual and hearing impairment can be isolating and may significantly affect communication, education, and daily activities. Diabetes can present in childhood, but more often presents in adulthood, requiring close regular monitoring and the potential need for insulin injections several times a day. Many complications, such as vascular disease, visual impairment, kidney disease and peripheral neuropathy are associated if the diabetes is not well controlled.

1.10. There is no cure for this condition and individuals are managed with multidisciplinary teams due to the diabetes, hearing loss and the loss of visual acuity.

1.11. The committee noted the executive's request to consider Wolfram-like syndrome (WLS), OMIM #614296, 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, Wolfram-like syndrome (WLS), OMIM #614296 is a serious, progressive neurodegenerative condition with symptoms present from early childhood. There is no cure for the condition which can lead to profound deafness, and severe visual impairment, with some of those affected being registered as legally blind during childhood or in early adult life. The condition may also include the development from a young age, of insulin dependent diabetes and its associated long-term complications. The committee considered the progressive nature of the syndrome and its potentially devastating physical and psychological impact on the quality of life of those affected.

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 that a person with such an abnormality will, given the condition's worst symptoms, have or develop a serious physical disability, a serious illness or any other serious medical condition.

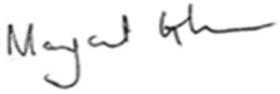
**2.3.** The committee was therefore satisfied that the following condition meets the criteria for testing under paragraph 1ZA(1)(b) and (2) of Schedule 2 of the Act. The committee agreed to authorise testing for:

- Wolfram-like syndrome (WLS), OMIM #614296

## 3. Chairs signature

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

**Signature**



**Name**
Margaret Gilmore

**Date**
23 December 2020