Questions & Answers on licence application DIR 181 – Clinical trial of a genetically modified Herpes virus for the treatment of cystic fibrosis

What does this licence allow?

Novotech (Australia) Pty Limited is conducting a clinical trial of genetically modified (GM) *Herpes simplex virus-1* as a treatment for cystic fibrosis. Cystic fibrosis is an inherited disease caused by a defect in the cystic fibrosis transmembrane conductance regulator (CFTR) gene. It affects the glands that produce mucus and sweat, and a thick, sticky mucus accumulates in the lungs, breathing passages and digestive tract. This often leads to serious lung infections and digestion problems. Cystic fibrosis sufferers who survive to adulthood live on average to their mid-forties, with death commonly due to lung failure.

The GMO is a gene therapy product intended to repair this genetic defect by restoring a functional copy of the CFTR gene to lung cells. It will be manufactured overseas and imported into Australia. Up to 15 people with cystic fibrosis are permitted to be treated with the GMO in a hospital setting, over a three year period.

How was the GM treatment designed?

The GMO is based on *Herpes simplex virus-1*, commonly associated with cold sores. It has been modified such that it cannot multiply after entering a cell and instead produces the CFTR protein. This is intended to replace defective CFTR found in patients with cystic fibrosis.

What is the purpose of the clinical trial?

The aim of the clinical trial is to gather data to assess the safety and effectiveness of the GMO in correcting the genetic defect in the lungs of cystic fibrosis patients.

What other regulatory processes apply to this trial?

Clinical trials must be conducted in accordance with requirements of the Therapeutic Goods Administration (TGA), which address the safety of trial participants. Before commencing, this trial will require approval by a Human Research Ethics Committee, and must be conducted in accordance with the *Guidelines for Good Clinical Practice*. Import of the GMO will also require approval from the Department of Agriculture, Water and the Environment.

What controls are imposed for this release?

The Risk Assessment and Risk Management Plan (RARMP) prepared for this application concluded that the clinical trial poses low to moderate risks to people and negligible risks to the environment. To manage these risks, licence conditions have been imposed requiring that trial participants be seronegative for HSV-1, be monitored over the course of the trial for primary HSV-1 infection and offered anti-viral medication if they acquire an infection. Effective protection of clinical trial staff from aerosolised GMO is also required. The licence also limits the number of trial participants, the types of facility where the trial may be conducted, and the duration of the trial. A range of additional controls are imposed to minimise the potential for exposure of people other than trial participants, and susceptible animals, to the GMO.

Want more information?

A number of documents relating to this decision are available on the DIR 181 page of the OGTR website or via Freecall 1800 181 030. These documents include the finalised Risk Assessment and Risk Management Plan (RARMP), a summary of the RARMP and the licence.

The Office of the Gene Technology Regulator

OGTR Website

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