Questions & Answers on licence application DIR 223 – Clinical trials of a genetically modified adenovirus for treatment of bladder cancer

What is this application for?

Ferring Pharmaceuticals Pty Ltd is seeking approval for clinical trials of a genetically modified (GM) adenovirus, nadofaragene firadenovec, for treatment of bladder cancer.

The GM adenovirus would be manufactured overseas and imported into Australia. It would be administered to up to 13 patients with non-muscle invasive bladder cancer (NMIBC) at clinical trial sites and hospitals in New South Wales and Victoria.

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, the trials would require ethics approval, and must be conducted in accordance with the *Guidelines for Good Clinical Practice*. Import of the GM human adenovirus treatment will also require approval from the Department of Agriculture, Fisheries and Forestry.

How has the GM human adenovirus been modified?

The GM adenovirus is a human adenovirus which has been genetically modified by removing DNA sequences to make it safe for patients and introducing a gene for a protein with anti-tumour effects. It can enter urothelial and tumour cells and produce the protein with antitumor effects within the affected cells.

What is the purpose of the trial?

The trial is to assess the efficacy of the GM adenovirus for treating patients with NMIBC, or patients with high-grade Bacillus Calmette-Guerin unresponsive NMIBC with carcinoma in situ (CIS) by administering the GM adenovirus alone or in combination with chemotherapy (gemcitabine and docetaxel) or immunotherapy (pembrolizumab).

Has the GM adenovirus been previously tested or used?

Clinical trials (Phases 1-3) of this GM adenovirus have been conducted in the United States (US). The US Food and Drug Administration also approved this GM adenovirus for bladder cancer treatment in 2022.

The commercial supply (including import, storage, transport, and disposal) of this GM adenovirus in Australia has been authorised under the licence DIR 217.

What controls are proposed for this release?

The consultation Risk Assessment and Risk Management Plan (RARMP) prepared for this application concluded that the clinical trial poses negligible risks to people or the environment. However, as this is a clinical trial under limited and controlled conditions, a number of licence conditions have been drafted to restrict when and where the trial can take place, limit the size of the trial, and restrict the spread and persistence of the GM adenovirus. For example, there are conditions relating to preparation and administration of the GM adenovirus and appropriate waste disposal. Full details of the draft licence conditions are available in the consultation RARMP.

How can I comment on this application?

The full consultation RARMP and a summary of the RARMP for application DIR-223 are available on the OGTR website, the consultation hub or via the contacts listed below. You are invited to submit your written comments (via the consultation hub or by email) on the consultation version of the RARMP, related to any risks to the health and safety of people or to the environment from the proposed clinical trial. Please note that issues such as **patient safety, quality and efficacy of a therapeutic products, and marketability and trade implications** do **NOT** fall within the scope of the evaluations conducted under the *Gene Technology Act 2000* as these are the responsibility of other agencies and authorities. Comments must be received by the close of the consultation period on **6 March 2026**.

What are the next steps in the evaluation process?

The RARMP will be finalised, taking into account submissions related to the protection of people or the environment. A de-identified summary of all comments received and consideration of those comments is included in the Appendices to the final RARMP. The finalised RARMP will inform the Regulator's decision on whether or not to issue a licence.

The Office of the Gene Technology Regulator

OGTR Website

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