Questions & Answers on licence application DIR 217 – commercial supply of a genetically modified therapeutic for bladder cancer treatment

What is this application for?

Ferring Pharmaceuticals Pty Ltd is seeking approval for the import, storage, transport, and disposal of a genetically modified (GM) therapeutic, nadofaragene firadenovec, as part of its commercial supply in Australia.

The applicant proposes to supply the GM therapeutic to urology and oncology departments of hospitals as a therapy for the treatment of adult patients with a certain type of bladder cancer, i.e. high-grade Bacillus Calmette-Guérin (BCG)-unresponsive non-muscle invasive bladder cancer.

The GM therapeutic contains a GM virus, which requires the applicant to seek review from both the Gene Technology Regulator and the Therapeutic Goods Administration before it can be used in Australia. Other regulatory requirements may also apply.

How has the GM therapeutic been modified?

The GM therapeutic 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 make the protein with antitumor effects within the affected cells.

Has the GM therapeutic been previously used?

This application is for the first approval of the GM therapeutic in Australia.

Internationally, the GM therapeutic has been approved by the Federal Drug Administration in the USA with the brand name Adstiladrin for the treatment of BCG-unresponsive bladder cancer.

What steps have been undertaken with respect to this application?

The Gene Technology Regulator has prepared a consultation Risk Assessment and Risk Management Plan (RARMP), which finds that the proposed commercial supply of this GM therapeutic poses negligible risk to the health and safety of people or the environment. However, licence conditions drafted in the consultation RARMP ensure that there is ongoing oversight of the commercial supply.

How can I comment on this application?

The full consultation RARMP and a summary of the RARMP for application DIR 217 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 commercial supply.

Please note that issues such as patient safety, quality and efficacy of a therapeutic products 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.

Written comments must be received by the close of the consultation period on **18 September 2025**.

What are the next steps in the decision-making 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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