



Summary of Licence Application DIR 229

Krystal Biotech Pty Ltd has made an application under the *Gene Technology Act 2000* (the Act) for Dealings involving the Intentional Release (DIR) of genetically modified organisms (GMOs) into the Australian environment.

Project Title	Commercial supply of beremagene geperpavec (B-VEC) treatment for wounds in patients with dystrophic epidermolysis bullosa (DEB).
Parent organism	Herpes simplex virus type 1 (HSV-1)
Genetic modifications	
Introduced/deleted genes	Insertion of the human <i>COL7A1</i> gene to allow expression of collagen Deletion of two viral genes, to remove the ability of the virus to replicate
GMO production method	Commercial GMP manufacture overseas
Principal purpose	Commercial supply of B-VEC for topical treatment of DEB
Previous authorisation	DNIR-707
Proposed location	Australia-wide
Proposed period of release	Ongoing from issue of licence

Background

Dystrophic epidermolysis bullosa (DEB) is an incurable, rare, genetic skin disorder caused by mutations in the *COL7A1* gene encoding the collagen alpha-1(VII) chain protein (COL7). COL7 is a structural component of anchoring fibrils, which hold the epidermis and dermis of the skin together and are essential for maintaining skin integrity.

The applicant proposes to commercially supply Beremagene geperpavec (B-VEC), a herpes simplex virus type 1 (HSV-1)–based topical gene therapy treatment for DEB, that aims to temporarily restore functional COL7 protein through delivery of the human *COL7A1* gene. B-VEC has previously been approved by the FDA, EMA, and in Japan as well as under DNIR-707.

Next steps

The Gene Technology legislation sets out what the Regulator must do, as well as what the Regulator can or must consider, before deciding whether or not to issue a licence for this application.

After seeking advice from prescribed experts, agencies and authorities, the Regulator’s staff will prepare a consultation version of the Risk Assessment and Risk Management Plan (RARMP) considering aspects of the application in accordance with the legislation.

The Regulator will seek comment on the consultation RARMP from the public, as well as a wide range of experts, agencies and authorities. The public and experts will be invited to provide submissions on the risks to human health and safety, and on risks to the environment from the proposed release.

At this stage, the consultation RARMP is expected to be released for comment in March 2027.

After consultation, the Regulator’s staff will finalise the RARMP, taking into account advice on relevant matters. The finalised RARMP will form the basis of the Regulator’s decision whether or not to issue a licence. The consultation and final versions of the RARMP and associated documents will be available on the [OGTR website](#) when they are released.

Other regulatory approvals

The proposed commercial supply must meet Therapeutic Goods Administration requirements, and the proposed import would need approval from the Department of Agriculture, Fisheries and Forestry (Biosecurity) prior to commencement.