16 March 2021

Office of the Gene Technology Regulator (MDP 54)  
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Canberra ACT 2601

[ogtr@health.gov.au](mailto:ogtr@health.gov.au)

RE: National Gene Technology Scheme, Consultation Regulation Impact Statement (CRIS)

Thank you to the Department of Health, Aged Care and Sport for the opportunity to provide a submission to the ‘National Gene Technology Scheme, Consultation Regulation Impact Statement’.

# The Rare Disease Industry Working Group (RDIWG)

The RDIWG is comprised of companies that share a common interest in rare disease treatments and orphan drug development including gene therapies. The companies that are represented here develop, manufacture and supply innovative rare disease treatments to the Australian rare disease community.

The purpose of the RDIWG is to collaborate to ensure that Australians with rare and ultra-rare diseases have timely and equitable access to treatments by:

1. engaging in policy discussions to ensure timely and sustainable access to rare disease treatments, and
2. ensuring that reimbursement procedures and guidelines are fit-for-purpose.

Within the RDIWG, there is a sub-committee focusing on cell and gene therapies, for companies with an interest in this space. This submission is being made on behalf of this Sub-Committee.

Gene Therapies represent an exciting and rapidly evolving mode of precision medicine representing potentially curative and/or disease modifying solutions to patients suffering from rare genetic disorders.

Australia remains a priority destination for international multi-centre clinical trials, including Gene therapies where trials are already ongoing in Australia.

To ensure that Australians suffering from rare genetic disorders have timely access to Gene Therapy clinical trials, a streamlined, risk-based approach to assessing and approving these technologies for use in clinical trials, is vital (this is discussed in more detail overleaf).

Under the current proposed consultation, The RDIWG’s position is that the Status Quo (Option A) is no longer fit for purpose. The recent Australian House of Representatives Standing Committee on Health, Aged Care and Sport – Inquiry into approval processes for new drugs and novel medical technologies in Australia, has received submissions that particularly highlight significant delays in access to Cell and Gene Therapies.

Considering the ongoing consultation, the RDIWG would like to make recommendations pertaining to Gene Therapies specifically in relation to clinical trials and subsequent regulatory review.

* **Recommendation 1: Update the Gene Technology Act and Gene Technology Regulations to reflect the rapid technological advances including emerging technologies.**

The current regulatory system is not appropriate for new technologies for Advanced Therapy Medicinal Products (ATMPs)/**Gene Therapies (GTs)**. ATMPs that contain GMOs which do not pose an environmental or biosafety risk and/or are very well understood by the OGTR through significant evaluation experience, should be exempted from GMO full evaluation requirements. This includes GTs using non-pathogenic and replication-incompetent vectors such as adeno-associated virus (AAV) vectors which are replication deficient because the modified recombinant vector has a complete deletion of the wild-type viral genes. For this reason, AAV vectors should neither be classified as “living organisms” nor be required to meet GMO full evaluation requirements, which add considerable time and complexity to the review process, resulting in delays for Australian patients to access potentially life-saving treatments.

* **Recommendation 2: Streamline the Regulatory Process through collaboration and information sharing between the OGTR and TGA**.

In Australia, ATMPs that contain GMOs must be appropriately authorised under both the Gene Technology Act 2000 and the Therapeutic Goods Act 1989, by the OGTR and TGA respectively. Evaluation by each agency involves significant overlap in consideration of health and safety. Both agencies also monitor post-marketing commitments and ensure mandatory reporting of additional information that may change an ATMP’s risk-benefit profile. However, each agency’s review and approval process is currently separate and independent. Given the significant overlap between the required evidence for the applications, the questions that arise from each agency, and potential post-marketing commitments, it would follow that better coordination, transparency and collaboration between the agencies would improve efficiency, thereby reducing duplication of efforts and avoiding delays in patient access. We therefore recommend the development of a pathway which enables inter-agency co-operation and information sharing whilst respective assessments of a new ATMP are conducted. This may include creating an option for sponsors to seek pre-submission advice at a meeting attended by both the TGA and OGTR; alignment of milestones such as evaluation reports, questions, and post-market requirements; and the opportunity, if warranted, for direct three-way discussions between sponsors, TGA and OGTR during the course of their reviews to avoid duplication of effort and increase efficiency.

* **Recommendation 3: Drive alignment/harmonization with other global standards/agencies to ensure that the development and approval of ATMPs and GTs are not delayed.**

Achieving global harmonization of the GMO Application Process through open dialogue with other regulatory agencies to share experiences and information on the development of ATMPs/GTs/GMOs is imperative for timely and expedited approval and access. Where comparable overseas regulators have completed a thorough risk-assessment prior to authorising clinical trials, the OGTR may consider exemptions for Australia, similar to existing TGA COR pathways. Similarly, leveraging Work Share and Global Reliance as outlined in the World Health Organization (WHO) [Good Reliance Practice](https://nam02.safelinks.protection.outlook.com/?url=https%3A%2F%2Fwww.who.int%2Fmedicines%2Fareas%2Fquality_safety%2Fquality_assurance%2FQAS20_851_Rev_1_Good_Reliance_Practices.pdf%3Fua%3D1&data=04%7C01%7CKEvans%40bmrn.com%7Cca6534c9e8cd4b7b869108d8d3fcf8a8%7C7a1fa97c71fe4f42a823eab0b6012858%7C0%7C0%7C637492430779780936%7CUnknown%7CTWFpbGZsb3d8eyJWIjoiMC4wLjAwMDAiLCJQIjoiV2luMzIiLCJBTiI6Ik1haWwiLCJXVCI6Mn0%3D%7C1000&sdata=V9mHN8jBPDFXWcYI38hEHZUv7TOTjGR4N%2FNdS2BkfME%3D&reserved=0) in Regulatory Decision Making for Medical Products (GRelP document)  or expanding the scope of Australia – Canada – Singapore – Switzerland – United Kingdom, (Access) Consortium to include ATMPs/GTs/GMOs, will promote efficient use of resources and mitigate delays in the development of GTMPs which may result in quicker access to potentially life-saving treatments.

# In summary

The RDIWG Sub-Committee on Cell and Gene Therapies welcomes the opportunity to work with the Australian Government and other stakeholders on fit-for-purpose reforms to the current regulatory procedures and guidelines so that Australians have safe, rapid and sustainable access to rare disease treatments, including gene therapies.

Yours sincerely

**Submitted by the cell and gene therapy subcommittee of the Rare Disease Industry Working Group (RDIWG), comprising:**

* **Biogen**
* **BioMarin**
* **Pfizer**
* **PTC Therapeutics**
* **Takeda**