Australian Government

Department of Health, Disability and Ageing

Proposed amendments to the Gene Technology Regulations 2001

CONSULTATION QUESTIONS

Register your details (mandatory questions)

What is your name?
[redacted]
What is your email address? [redacted]
Question: Are you submitting a response on behalf of your organisation or as an individual?

- Organisation
- Individual

What is your organisation? [redacted] is the peak body representing the research-based pharmaceutical industry in Australia.

Key changes of note

1. Do you have any comments or concerns with regards to the proposed changes to the structure of the GT Regulations generally?

   Response: [redacted] is generally in support of the GT Regulations. Aligning the Regulations with the amended Act will improve navigability. In order to ensure no disruption to ongoing CTN trials across clinics and hospitals, our members would like a **concordance table** to be published. This should map current provisions to new numbering so that our members can rapidly update their SOPs, clinical governance materials and site training packs.

Part 1 – Preliminary Definitions

2. Do you consider that any other terms are unclear and/or require definition?

   Response: Broadly clear and appropriate for clinical use.

   Specifically, for CTN prescription-medicine gene therapies:

   "Therapeutic dealing"—confirm explicit coverage of **in-vivo** administration (e.g., intravitreal AAV; systemic infusions) in clinical settings to align with TGA's treatment of gene therapy delivered in humans as **prescription medicines.**
   "Contained dealing"—add an interpretive note that **clinical environments** (wards, theatres, day surgery suites, infusion units) are considered contained when operating under the **Regulator's Rules** for transport, storage, disposal (TSD) and any permit conditions, so clinics/hospitals can implement standard procedures without ambiguity.
   "Novel trait"—provide therapeutic examples (e.g., a new promoter/regulatory switch or a new nuclease affecting tropism or persistence) to support consistent IBC classification across clinics and hospitals.

   "High risk GMO" that would require consultation with GTTAC and have a longer timeframe for assessment (90 days currently vs. 120 days). It could be argued that adeno-associated virus-based gene therapies are a well-established technology that wouldn't be considered "high-risk" for a commercial license. Moreover, the Regulations are proposing a 30-day evaluation period for clinical trials for adeno-associated virus-based gene therapies (under certain circumstances) so it

seems inconsistent that a 90/120-day period would be required if the gene therapy was to be transported, stored and administered in the same way in the commercial setting.

Part 2 – Interpretation and General Operation

3. Are you satisfied with the proposal of certain risks being excluded from the requirement of ministerial and Regulator consideration if they are already considered under another scheme?

Response: Yes. For CTN gene therapy, HREC conducts scientific risk review while the TGA is notified (no scientific assessment under CTN). [redacted] supports avoiding duplication by the OGTR of risks already addressed via HREC under CTN, consistent with the consultation's intent to avoid overlapping risk review across regulators.

[redacted] requests that a simple OGTR–TGA–HREC responsibility matrix clarifying
(i) patient-level risks in CTN (HREC),
(ii) product-level risks in CTA (TGA), and
(iii) biosafety/environmental controls in permits/Rules (OGTR)

is published.

Part 3 – GMO licences

4. Do you consider the concept of a designated dealing clear?

Response: Mostly. Please add therapeutic examples distinguishing designated vs permit-eligible dealings e.g. replication-competent vectors, constructs with plausible generation of infectious agents, or features that materially elevate environmental persistence, to help IBC, clinics and hospitals classify consistent.

5. Do you have any concerns with the proposed consultation process for RARMPs?

Response: [redacted] supports a risk-proportionate consultation. For contained clinical trials in clinics/hospitals, public consultation should occur only where the GMO introduces novel, non-contained environmental risks. [redacted] suggests a published decision flowchart indicating when GTTAC/states/public are consulted (vs targeted consultation only), tied to novelty/persistence criteria.

6. Do you have any concerns with revised timeframes?

Response: [redacted] supports the alternative consideration periods (e.g., shorter where no consultation), with extended timeframes reserved for genuinely complex categories (e.g., gene drives with release). For clinical, contained P-class permits used in CTN trials, we recommend operationalising the shortest period via Rules and Standard Conditions to provide predictable first-patient-in timelines for applicants, clinics and hospitals.
Ideally, the frequency of GTACC meetings for consultation would be increased to ensure the 90 day timeline was maintained and that there were not extensions of application review timelines.

7. Do you have any concerns around the proposed range of dealings that will be required to be licensed?

Response: [redacted] supports this, with a request to publish the scientific rationale distinguishing licence vs permit/ND/NND for therapeutic dealings, to prevent over/ or under-classification across varied clinical sites (e.g. eye clinics vs hospitals).

Part 3 – GMO Permits

8. Do you have any concerns with dealings that are proposed to be authorised by a GMO permit?

[redacted] supports P2 (clinical vectors) and P4 (GM somatic cell introduction) as the main pathways replacing DNIR for clinical gene therapy. To ensure feasibility across small eye clinics (intravitreal AAV) and hospital infusion units:

1. Avoid full-site accreditation burdens for clinical-only administration. The consultation paper's ND classes require that the person undertaking the dealing be an accredited organisation; by extension, many read permits the same way. This is unworkable for small clinics working towards mature research governance/IBC infrastructure, yet the clinical acts remain contained under controlled SOPs. We recommend allowing clinical-only sites to operate under the sponsor's accreditation (preferred) or via a streamlined "clinical authorised site" mechanism (no full accreditation), provided they implement the Regulator's Rules and permit conditions for TSD, PPE, spills, and patient waste management.

2. Single national permit + notification activation. Issue one P2/P4 national permit per product and allow site activation by notification for any accredited organisation (or a clinical-authorised site under the sponsor's accreditation). This mirrors the notification logic used for pre-notified ND classes and leverages Rules for standardised conditions; it removes per-site permit variations and accelerates national roll-out across clinics and hospitals.

3. Publish standard P2/P4 templates in Rules. Provide national templates (dispensing, PPE, waste, vector shedding/incident reporting; optional facility criteria for infusion suites and theatres) so heterogeneous clinical sites (ophthalmology clinics vs infusion wards) can implement uniformly on day one.

Part 3 – Notifiable dealings

9. Do you have any concerns in relation to the proposed notifiable dealings classes?

Response: [redacted] supports the post-notified contained classes modernizing NLRDs and the pre-notified classes where other regulators manage substantial risk. We note ND1-ND3 require the person undertaking the dealing to be an accredited organisation; this is appropriate for lab support/QC (PC2 certified labs) but is not workable for clinical-only sites.

[redacted] requests that you:

- Clarify that ND accreditation applies to lab-based dealings (e.g., vector QC, sample handling), not clinical administration; and/or

- Expand "person engaged by the accredited organisation" to include contracted clinical-only sites operating under the sponsor's accreditation and permit conditions, thus removing the need for the clinic to seek full OGTR accreditation independently.

- Validate Records of Assessment (RoAs) for a minimum five-year period, allowing for administrative revalidation of unchanged RoAs to avoid unnecessary IBC assessments for routine methods in hospitals or clinical laboratories.

Part 3 – Non-notifiable dealings

10. Do you have any concerns in relation to the proposed non-notifiable dealings classes?

Response: [redacted] supports this. NND1–NND4 appropriately capture low-risk contained laboratory functions supporting clinical studies (e.g. limited QC). Clarify any viral-vector restrictions for NND4 (GM human somatic cell introduction) to avoid ambiguity where hospitals handle gene-modified cells in separate programs.

11. Do you consider the language 'not involving intentional release into the environment' appropriate for NNDs?

Response: Yes. Clinical administration in clinics/hospitals is contained, and humans are not GMOs under the proposed definitional approach; the phrase maintains the boundary between contained clinical use and environmental release scenarios.

[redacted] ***also has the following further proposals for consideration:***

Please provide a draft of the of the proposed updated Regulations to allow our members to review and understand them in their totality.

Additional Accelerators tailored to clinics and hospitals under CTN

1. Create an Established-Vector Clinical Stream within P2/P4 for replication-defective vectors (AAV, non-replicating Ad, LVV rendered replication-incompetent, mRNA/LNP), with shorter consideration periods unless a novel trait/parent elevates risk; this fits the consultation paper's risk-tiering intent and maintaining efficient ophthalmology and hospital infusion services.
2. OGTR–TGA–HREC matrix for CTN trials: clarify that CTN = TGA notification only (no scientific assessment), HREC = scientific/ethical review, OGTR = biosafety/containment controls via permits/Rules; publish a simple one-page guidance to prevent duplicate requests from applicants, IBCs, clinics and hospitals.
3. Containment via Rules, not certification for clinical rooms. Clinical theatres/infusion suites generally should not require OGTR facility certification; instead, require compliance with Rules (TSD, waste, PPE) and any permit conditions. Lab-based activities remain in certified PC facilities per OGTR guidance (PC2/PC3).
4. Monitoring proportionality. As more organisations become involved, OGTR's risk-based monitoring should emphasize coaching/education during initial visits to new clinical-only sites (ophthalmology or infusion units) to support safe adoption without creating barriers to participation.
5. To support consistent, safe, and efficient compliance across clinical research settings, the OGTR could develop clear guidance and educational materials **for applicants**, outlining expectations for SOPs, site-level biosafety responsibilities, documentation requirements, integration with IBC, HREC and TGA processes and importantly, examples of acceptable SOP formats for varied clinical settings.

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Privacy information and consent to publish

Your personal information is protected by law, including the Privacy Act 1988 and the Australian Privacy Principles. It is being collected for the primary purpose of consulting with stakeholders on proposed amendments to the Gene Technology Act 2000 and the Gene Technology Regulations 2001. Your information may also be used and disclosed for other purposes, such as development of further consultation activities.

Please note that your email address will not be published and responses may be moderated to remove content that is inappropriate/offensive or contains sensitive information.

12. The Department of Health, Disability and Ageing, on behalf of the National Gene Technology Scheme (the Scheme), intends to publish submissions, including the name of the individual or organisation that made the submission, on the Scheme website. Please indicate your willingness for your details to be published by selecting the appropriate response below. The following question requires one answer to be selected.**(Required)**

    a) I CONSENT to the submission being published in full on the Scheme website.

    √ I CONSENT to publication of my anonymous submission on the Scheme website, but do not consent to any identifying information being published.

    b) I CONSENT to a redacted version of my submission being published on the Scheme website.

    c) I CONSENT to publication of the name I have provided in a list of submissions received on the Scheme website, but do not consent to any part of my submission being published.

    d) I DO NOT CONSENT to any information about my submission, including my name or the name of my organisation, being published on the Scheme website.

    **If any part of your submission is confidential information that cannot be cited, please clearly mark these parts "IN-CONFIDENCE" and they will be redacted for publication.*

13. By making a submission, I acknowledge that: **(Please select all that apply.)** √

    √ I understand that the giving of my consent is entirely voluntary.

    √ I am over the age of 18 years.

    √ I understand the purpose of the collection, use, publication or disclosure of any submission.

    √ I understand that, where I have provided consent to my submission being published, the Department has complete discretion as to whether my submission, in full or part, will be published.

14. Third Party personal information and evidence of consent to publish **(Please select all that apply.)**

    ☐ Please tick this box if your submission contains personal information of third-party individuals. You should not include personal information about third parties unless you are able to provide evidence of written consent.

    ☐ Please tick this box if you have attached evidence of written consent.

    √ Please tick this box if none of the above options apply.

    If you have indicated above that you have attached written evidence of third-party consent, please attach it here.