



Cell & Gene Catalyst response to the *Health Technology Assessment Policy and Methods Review - Consultation 2*

TO: Department of Health and Aged Care
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From: Cell & Gene Catalyst, a joint venture of AusBiotech and Medicines Australia

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About the Cell & Gene Catalyst

The Cell & Gene Catalyst (Catalyst) is a national joint venture of two industry peak bodies, AusBiotech and Medicines Australia. The Catalyst was established to grow and accelerate Australia's cell and gene therapy ecosystem and it is led by an Expert Steering Group from the Catalyst's industry partner organisations.

AusBiotech is the Australian representative body for one of Australia's most innovative industries with a well-connected network of over 3,000 members in the life sciences industry, which includes biotherapeutics, medical technology (devices and diagnostics), food technology and agricultural biotechnology sectors.

Medicines Australia (MA) leads the research-based medicines industry of Australia. MA members discover, develop and manufacture prescription pharmaceutical products, biotherapeutic products and vaccines that bring health, social and economic benefits to Australia. Members invest in Australian medical research and take local discoveries and developments to the world.

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The Cell and Gene Catalyst (the Catalyst) welcomes the opportunity to respond to the Department of Health and Aged Care's Health Technology Assessment (HTA) Policy and Methods Review - Consultation 2.

Establishing a streamlined access and treatment pathway for cell and gene therapies is essential and is the first policy priority for the Catalyst. Given Australia's current systems were not set up for these potentially revolutionary therapies, addressing this priority area together will enable health system readiness and industry growth, ensuring everyone in Australia will have access to these advanced therapeutics.

The Catalyst partners, Expert Steering Group and AusBiotech, endorse Medicines Australia's recommendations and responses to the HTA options paper in full.

As an entity enabling the growth, development and translation of cell and gene therapies, the Catalyst highlights key options below:

The Catalyst calls for the following options to be significantly strengthened:

- *Option 1.4: The Catalyst supports a nationally cohesive approach to HTA for technologies that are currently jointly funded, however considers the ideal way to simplify and streamline access pathways for cell and gene therapies is to both assess and fully fund these advanced therapeutics at the federal level.* Regular consultation, communication and collaboration with States and Territories will continue to be important for these products. To enable holistic consideration of product costs (therapy plus delivery), States and Territories would need to articulate the total costs of product delivery for inclusion as inputs into the HTA process from the beginning.
- *Option 3.3: Reducing the discount rate not only recognises the value of long-term future benefits – it also brings Australia in line with international best practice.* Cell and gene therapies are designed to have a lifelong, potentially curative impact. Treatment involves an upfront cost followed by potentially lifelong benefits for the patient, their family and society in general. Australia's high discount rate means our HTA system is ill-equipped to adequately value the lifelong benefit these treatments can offer.
- *Option 4.3: To ensure equal and equitable access to cell and gene therapies, a high-level principles-based framework for accepting and assessing real world evidence, non-randomised control trial evidence and surrogate health outcomes is required.* Conditions treated by cell and gene therapies can be rare or extremely rare which means they can have small patient cohorts in Australia. This means recruiting participants for clinical trials is more challenging and generating randomised control trial evidence more difficult, emphasising the value of RWE. Real world evidence (RWE) and broader societal, health and economic benefits can be transparently considered and used in HTA decision-making – potentially leading to faster, more equitable patient access.

There are significant health and economic opportunities with cell and gene therapies, and advanced therapeutics more broadly. Working together, Australia can successfully navigate the barriers and leverage the opportunities – and the cell and gene therapies ecosystem is ready.