Fact Sheet:
Cell and Gene Therapies

Medicines Australia

The HTA Review makes multiple recommendations, which, when implemented as a package, will improve processes, accountability and timelines for highly specialised therapies such as cell and gene therapies.

The HTA Review recognises the opportunities for health benefits, presented by advances in medical science, are significant, and that health technologies emerging today are increasingly diverse, complex, and expensive. It acknowledges the role of HTA to enable governments to deliver equitable, universal and best possible health care is becoming more important than ever, and there is both need and opportunity to significantly improve timely access to important, and transformative health technologies.

Recommendation 13 proposes improved processes, accountability and timeliness for Highly Specialised Therapies (HST) typically delivered in hospitals and co-funded by State / Territory Governments and the Commonwealth. This includes:

- Expediting the development and implementation of the nationally cohesive approach to Health Technology Assessment (HTA) as outlined in Schedule C of the 2020-2025 National Health Reform Agreement (NHRA) Addendum.
- A national HTA framework including processes for HTA to inform advice on implementation, investment and disinvestment opportunities at Commonwealth and State levels, leveraging work already underway through the Health Technology and Genomics Collaboration (HTGC).
- A methodology, developed and implemented in consultation with stakeholders, to consider the cumulative impact of high-cost HST's on the health system, at a national level.
- The development of criteria to ensure a nationally consistent process for patient selection and allocation for HST's.
- Establishing timeframes for implementing high-cost HSTs, funded through the NHRA Addendum, where the therapy has a positive HTA recommendation and modelled on targets agreed with respect to the timeframes for listing medicines on the PBS.
- Within three months of the in-principle pricing agreement, a national-level implementation plan to be published in collaboration with state and territory governments with timelines for implementation in the different jurisdictions, and details of how the state and territory governments will ensure patients can access the treatment within six months of in-principle pricing agreement.
- A nationally cohesive approach to HTA, transparency on the HTA process following a HTA recommendation, and the establishment (or participate in existing international collaboration) of a horizon scanning process to identify, prioritise, assess and monitor high cost HST's funded through the NHRA Addendum.

Other recommendations that will accelerate access to Cell and Gene therapies include:

- A framework that supports the use of different contract and funding mechanisms to subsidise health technologies, aside from the standard 'price per unit' approach which addresses the implications of high cost/high impact health (Recommendation 16).
- A unified pathway for all health technologies that will remove uncertainty about PBAC or MSAC assessment of Cell and Gene therapies (Recommendation 4).
- The revised policy for Managed Entry Arrangements (Recommendation 19).
- Proposed Bridging Fund (Recommendation 20).
- Reforms to enhance real-world data and real-world evidence (Recommendations 27 – 31).
- A lower the discount rate to 3.5% for health technologies with high upfront costs and long-term benefits (Recommendation 39) that will help capture the full value of these products.
- A framework for PICO development (Recommendation 32).
- Updating guidelines for assessment of consumer evidence (Recommendation 33).
- Methods for non-randomised and observational evidence (Recommendation 35).
- Methods for assessment of surrogate endpoints (Recommendation 36).
- Horizon Scanning (Recommendation 47).

The Review recognises that many cell and gene therapies will be launched in Australia soon. Implementation of these reform recommendations will address existing barriers to early and equitable access of these therapies.