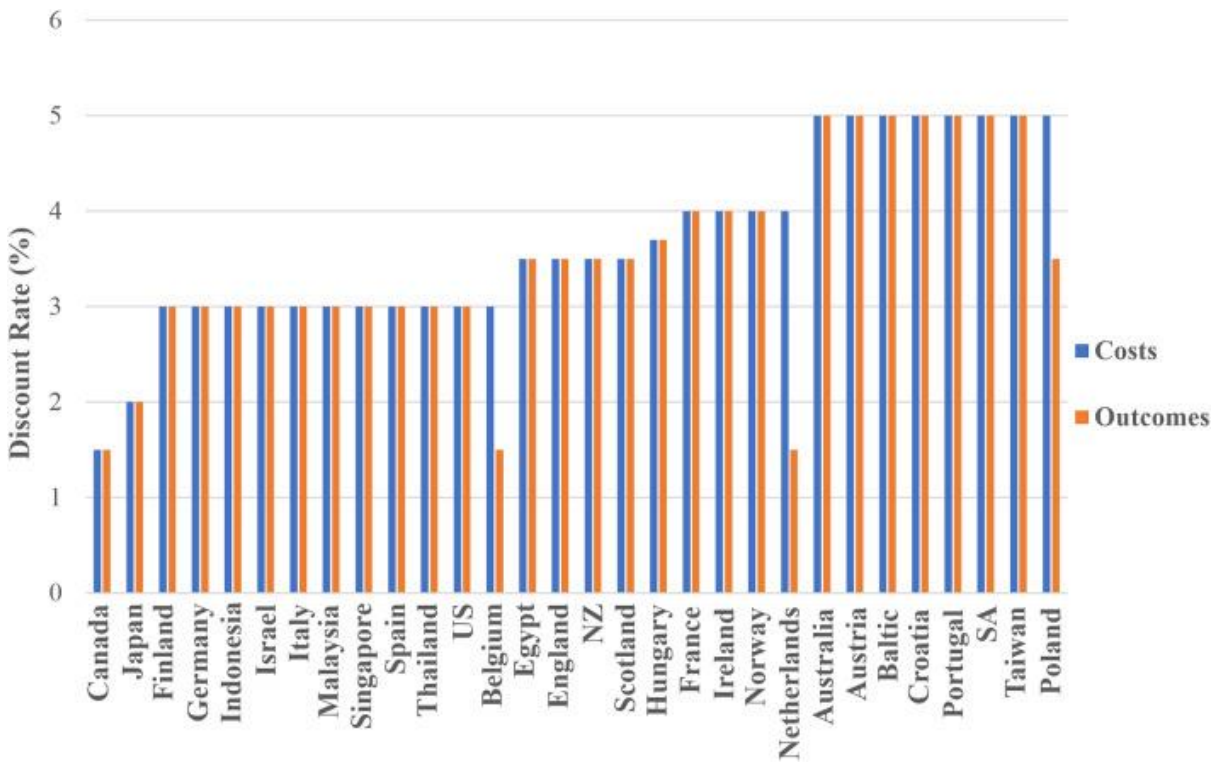
How does the discounting method in section 3A.1 of the PBAC guidelines compare with discounting methods used in economic evaluations that support other public funding decisions in Australia and in comparable overseas jurisdictions?

The current discounting rates are significantly higher than other countries, including countries with comparable Health Technology Assessment systems such as Canada, New Zealand, and England, suggesting that an adjustment to align Australia's discount rate with international best practice would be appropriate.



Discount rates recommended by national healthcare economic evaluation guidelines. *SA* South Africa[1]

Does the base case discount rate outlined in section 3A.1 of the PBAC guidelines need to be changed? If so, what should it be and why?

Background

There are limited treatment options for rare diseases, so it is essential that people living with a rare disease can benefit from new and transformative health technologies. As many rare diseases are progressive, time is often critical. Policy, including HTA policy, must accelerate timely and equitable reimbursement of new medicines and emerging technologies. Australian HTA processes utilise models that are designed primarily for more common diseases. This presents challenges for reimbursement decisions for medicines/technologies for rare diseases. Smaller patient numbers impact cost effectiveness and this highlights the importance of fit-for-purpose approaches to HTA models for rare diseases. The PBAC discount rate presents an opportunity to adjust policy settings to address the challenges and inequities currently faced by rare disease patients.

In 2014, an Australian report found that Australians generally gain access to rare disease therapies anywhere between two to four years after comparable countries such as the United Kingdom, Canada, Germany and the Netherlands[2]. Through RVA's work with our Round Table of Companies, a group of pharmaceutical companies with a common interest in rare diseases and orphan drug development, we know that internationally, the pharmaceutical industry's general perception is that Australia is a challenging market with uncertain approval processes. As such, the Australian market is often allocated lower priority by international companies. This causes great despair, frustration, and confusion to Australian rare disease patients when they see that 'their' medicines are available in other countries but not here in Australia.

The National Strategic Action Plan for Rare Diseases 2020[3] identified equitable access to health technology and fit-for-purpose reimbursement pathways as key priorities:

Priority 2.4 *Enable all Australians to have equitable access to the best available health technology.*

Action 2.4.1 *Develop policy that supports people living with a rare disease to have timely and equitable access to new and emerging health technologies.*

Action 2.4.2 *Ensure funding and reimbursement pathways are fit-for-purpose and sustainable for current and new health technologies for rare diseases.*

Recommendations

Recommendation 1

The current base discount rate of 5% be reduced to align with international best practice in comparable international jurisdictions such as Canada, New Zealand, and England

Recommendation 2

Lower and differential discount rates be applied to rare disease therapies to make current HTA more fit for purpose in assessing rare disease therapies and to bring Australia into alignment with international best practice and WHO recommendations.

The current discount approach means that therapies that deliver benefits over the long term are considered less cost effective.[4] This disproportionately impact on evaluation of cost-effectiveness for rare disease therapies because so many rare diseases have childhood onset and are chronic requiring ongoing treatment or because emerging therapies that have the potential to be transformative for rare genetic disorders such as cell and gene therapies have high initial costs with longer term benefits.

The 2021 white paper "Cell and Gene Therapies: Rising to the Challenge"[4] identified several examples of discounting practices in international jurisdictions that apply lower and differential discount rates to therapies where there are health benefits over a prolonged period and for therapies such as cell and gene therapies that have initial costs but can be transformative.

"The Netherlands, Poland and Belgium specify differential discounting in their HTA processes such that health gains, or effects, have lower discount rates than costs. Belgium justifies this differential discounting (1.5 per cent for effects) to avoid penalising interventions that deliver most of their benefits in the future (8, 9). The Netherlands apply a 1.5 per cent for effects noting that the value of health has increased over time (9). New Zealand's 3.5 per cent discount rate for both costs and effects is based off actual social rates of time preferences (from the long-term, government bond rate) (9). The basis for Australia's 5 per cent discount rate remains unknown and has not changed since 1995 (14).

Although they traditionally use a 3.5 per cent discount rate for costs and effects, the UK's HTA agency NICE applies reduced discount rates in specific circumstances where long-term benefits (>30 years) are anticipated (14, 15). Recently, this was the case for a paediatric oncology drug whereby NICE decided to apply differential discounting under the proviso that "treatment restores people who would otherwise die or have a very severely impaired life to full or near full health, and when this is sustained over a very long period (normally at least 30 years)." Specifically, NICE stated that health benefits after 30 years should be valued at 1.5 per cent per annum provided the benefit was "curative and substantial" (17). In their current methods review, NICE has also acknowledged that "there is also a case to change how NICE values costs and health effects for health technologies in the future (through discounting)" (15). Finally, the World Health Organisation (WHO) maintains that differential discounting and the use of non-constant discount rates should be applied when evaluating effects over long-time scales (7)"[4] p26

Rationale for Recommendation

- A change to the current base discount rate would align Australia with comparable international jurisdictions.
- A fit-for-purpose discounting rate for therapies for rare diseases addresses the priorities identified in the National Strategic Action Plan for Rare Diseases

- Differential discounting for rare disease therapies could address inequity currently experienced by Australians living with a rare disease in access the therapies and is aligned with international best practice and WHO recommendations.

References

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