



High-cost gene therapies and the law of the public purse:

who decides, who reviews and who pays?

SARA ATTINGER - USYD
CAMERON STEWART
IAN KERRIDGE
WENDY LIPWORTH



'Public Purse'

Simon Perry 1994

Source: <https://citycollection.melbourne.vic.gov.au/the-public-purse/>



Overview

1. The challenge of high cost gene therapies
2. Legal considerations for public funding: mapping the territory
3. Findings: what can law do and not do
4. Investigating how decisions are being made



1. High upfront cost gene therapies



eg Zolgensma, \$2.5 million per dose (one-time)

The screenshot shows a government media release page. At the top is the Australian coat of arms. Below it is a navigation menu with links: Home, Media centre, Mark Butler, Jenny McAllister, Sam Rae, Emma McBride, Rebecca White, and Dan Repacholi. The main content area has a dark background and features the following text: 'Life changing gene therapy for babies with spinal muscular atrophy'. Below the headline is a circular portrait of Mark Butler, followed by his name and title: 'The Hon Mark Butler MP, Minister for Health and Ageing, Minister for Disability and the National Disability Insurance Scheme'. A sub-headline reads: 'The Australian Government is giving more families access to life changing gene therapy for spinal muscular atrophy through the Pharmaceutical Benefits Scheme (PBS)'. At the bottom, it states 'Media event date: 23 October 2023'.



1. High upfront cost gene therapies

- 6 funded, at least 2 being considered by Aus Gov process currently
- Need a robust basis for funding decisions (as part of health technology assessment or 'HTA')
- MRFF grant (Prof Kirsten Howard, USyd):

Development of a generalisable evaluation framework for high upfront-cost gene therapies: clinical, financial, ethico-legal and cultural considerations



2. Mapping the territory

What are the legal considerations for public funding of high cost gene therapies?

1. Who decides?
2. How do they decide?
 - What can/must they take into account

→ What can law even do in this space?



2. Mapping the territory

- HTA review completed 2024
- No comprehensive map of ethico-legal considerations for HTA and public funding of therapies
- Focus here on the legal

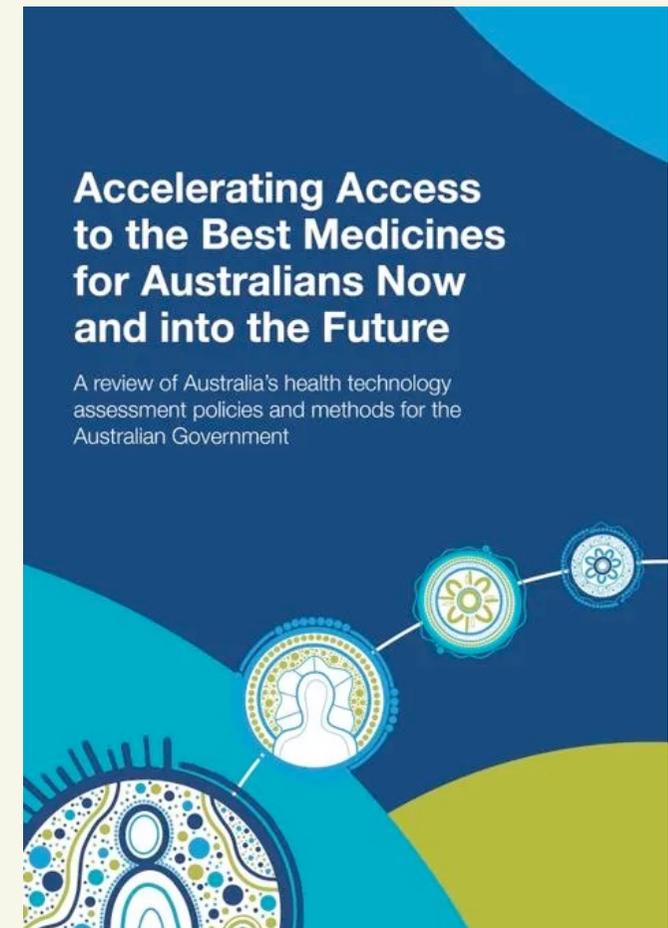
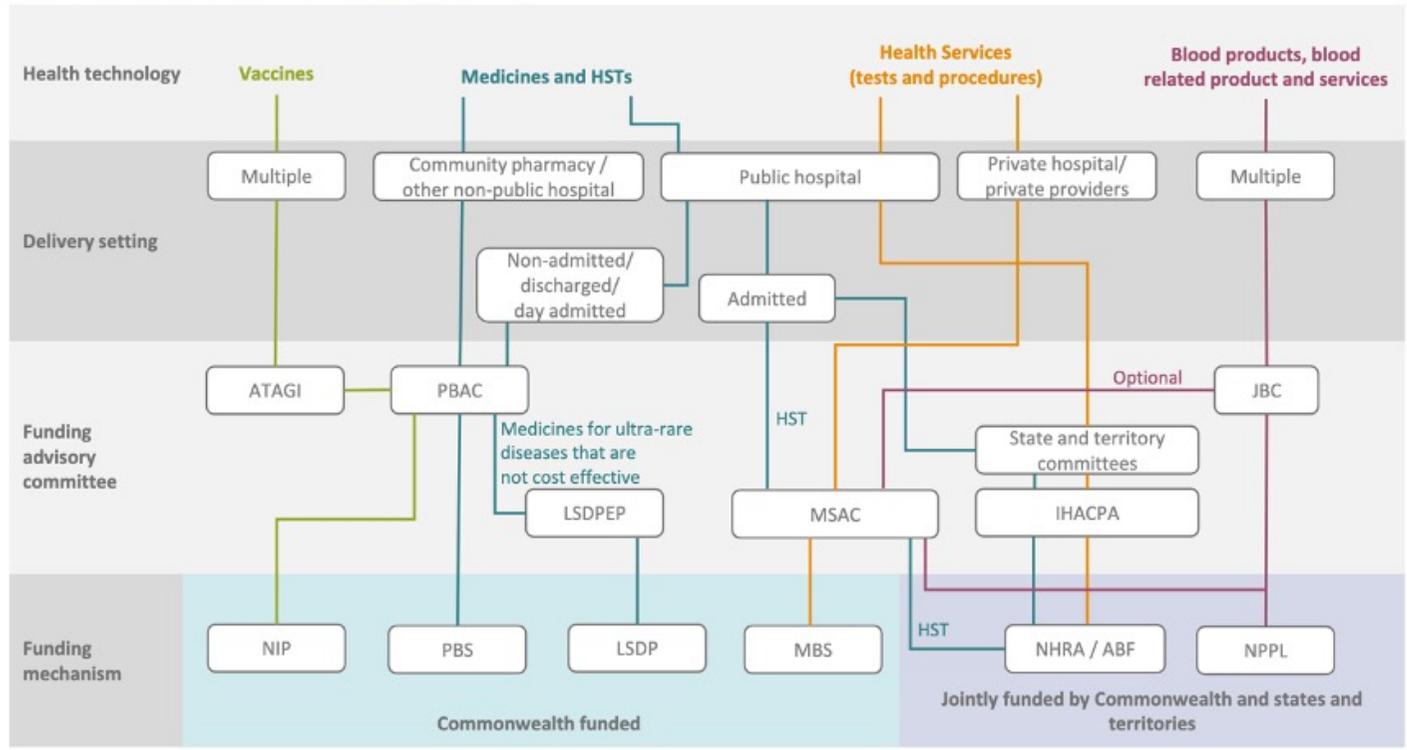


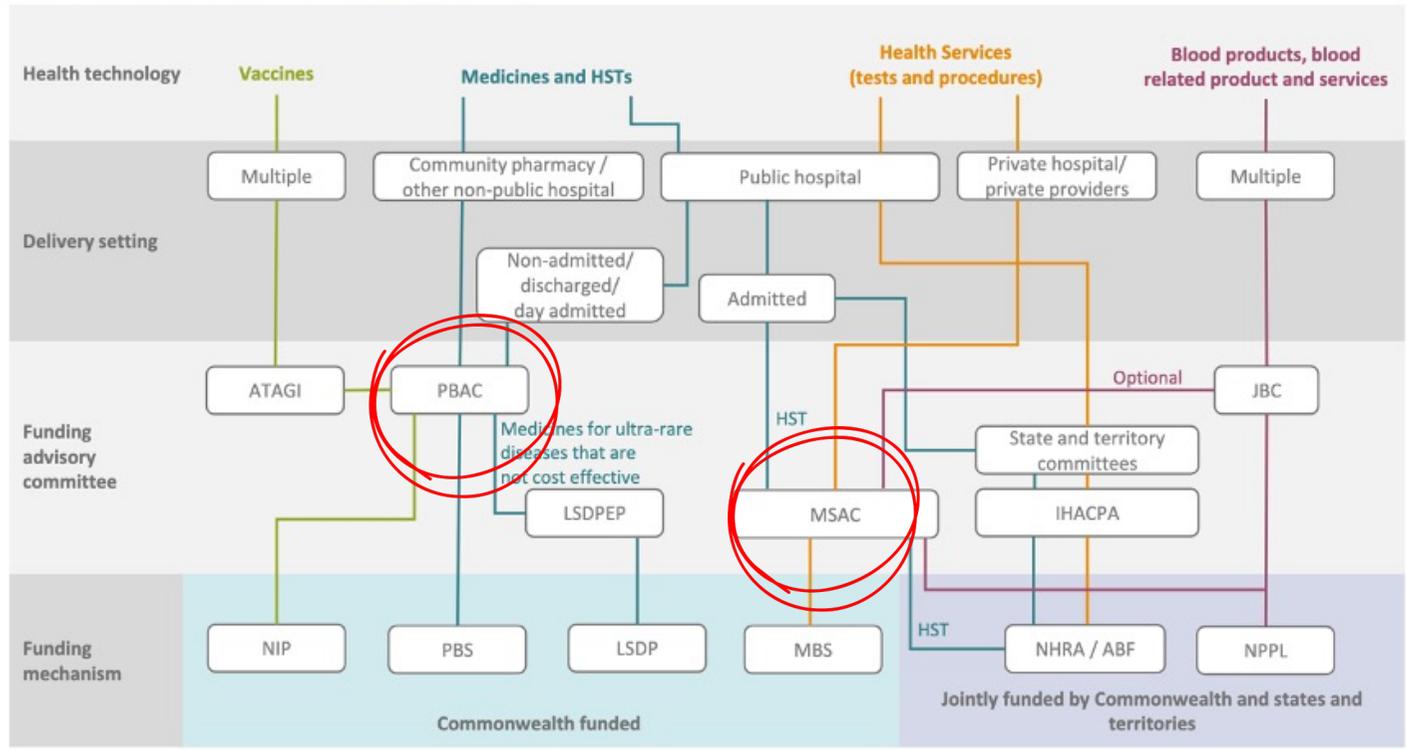
Figure 3. Funding pathways for Commonwealth funded health technologies



Acronyms: ABF = Activity Based Funding; ATAGI = Australian Technical Advisory Group on Immunisation; IHACPA = Independent Health and Aged Care Pricing Authority; JBC = Jurisdictional Blood Committee; LSDPEP = Life Saving Drugs Program Expert Panel; LSDP = Life Saving Drugs Program; MBS = Medicare Benefits Schedule; MSAC = Medical Services Advisory Committee; NIP = National Immunisation Program; PBAC = Pharmaceutical Benefits Advisory Committee; PBS = Pharmaceutical Benefits Scheme; NHRA = National Health Reform Agreement; NPPL = National Product Price List.

(Acronym city)

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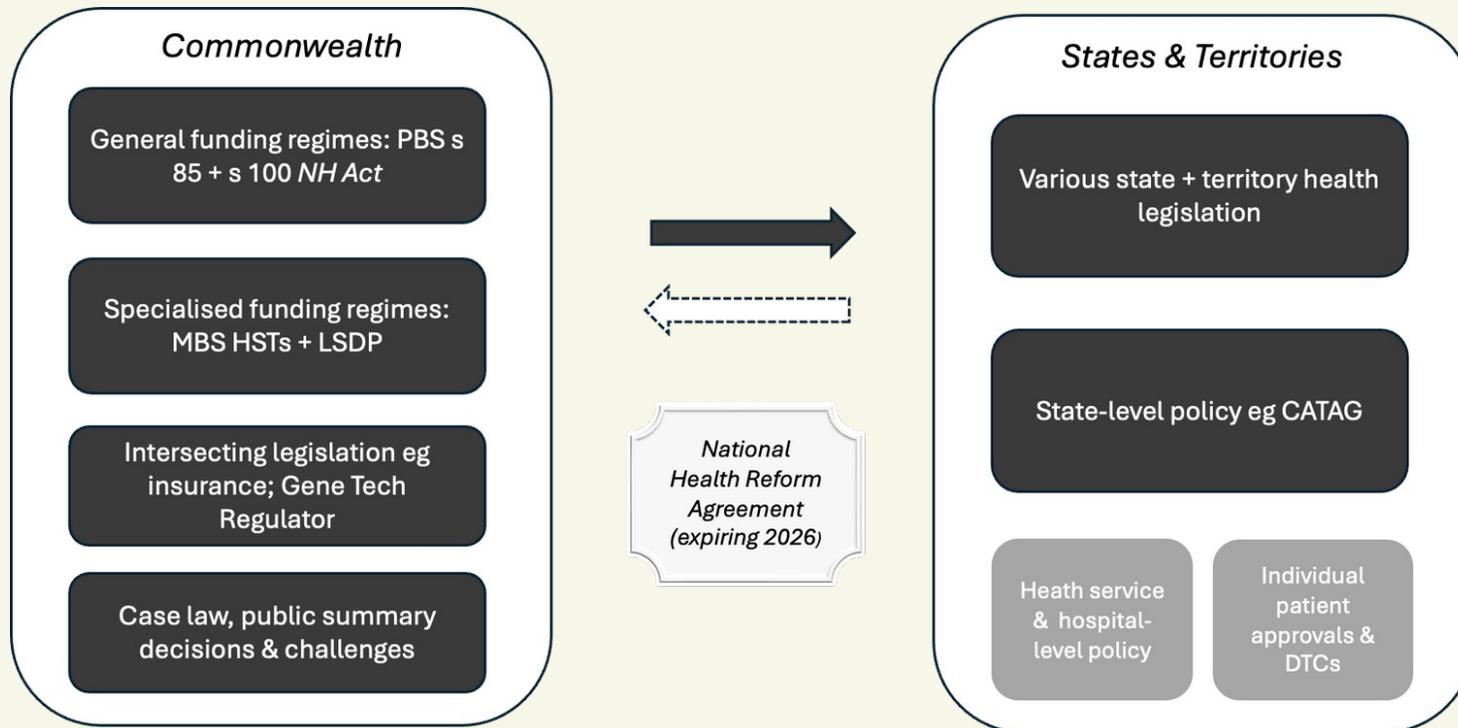
Medical Services

Pharmaceutical Benefits



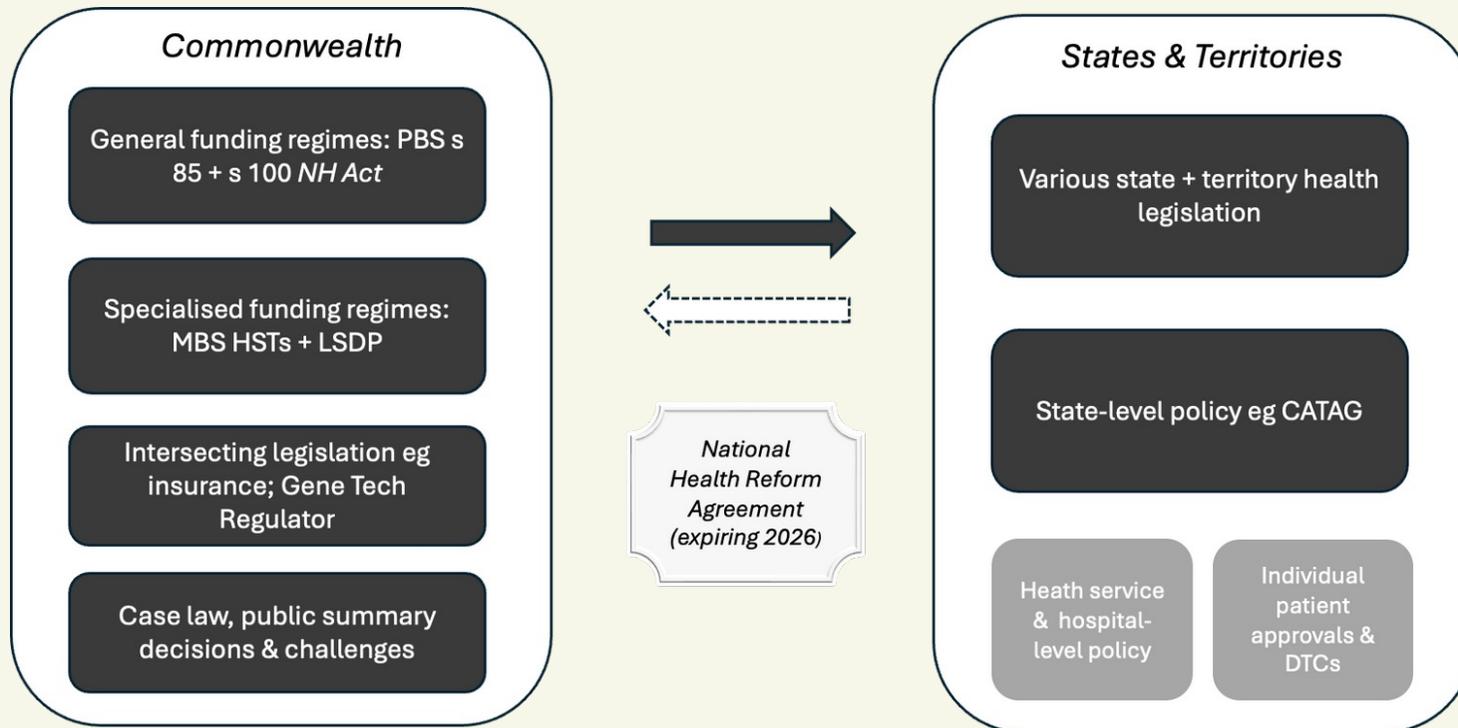


2. Mapping the territory



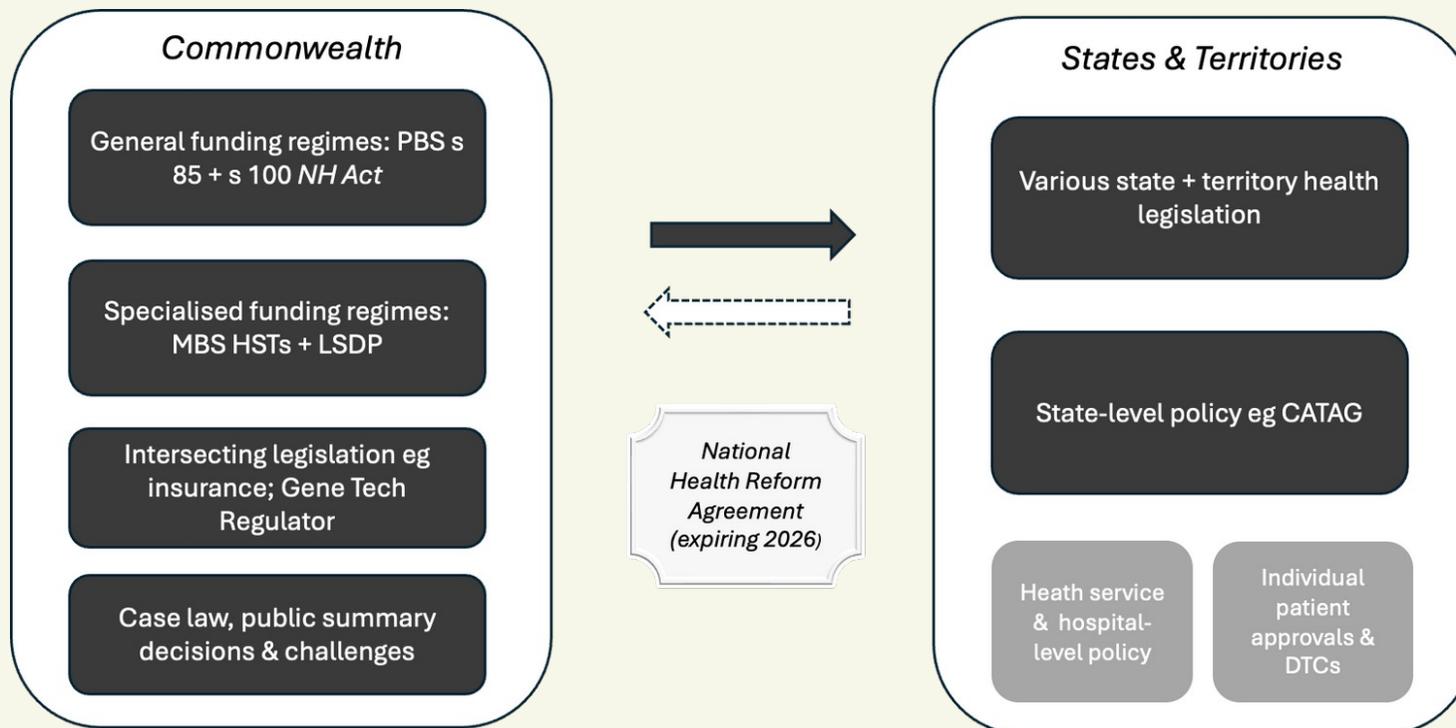


2. Mapping the territory



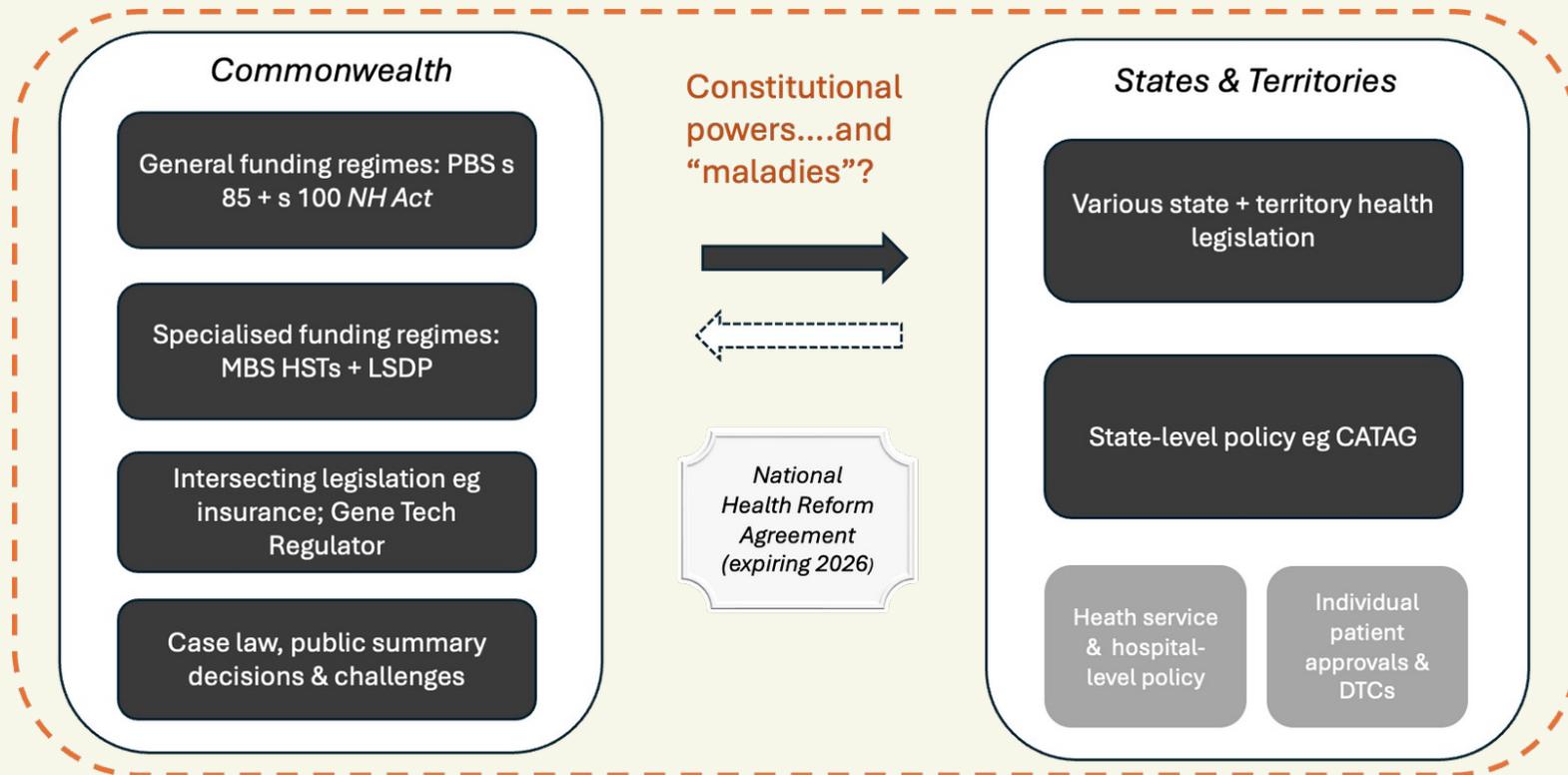


2. Mapping the territory: 'baggage'



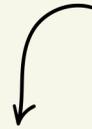


2. Mapping the territory: 'baggage'





2. Mapping the territory: how



Medical Services

Pharmaceutical Benefits



Non-statutory committee:
executive power to
perform an advisory role

Process, criteria and
constitution set out in
statute



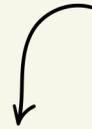
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Medical Services

Pharmaceutical Benefits



Inherent discretion (executive power)

*Can take into account broader considerations:
Pfizer Pty Ltd v Birkett (2001); GlaxoSmithKline
Australia Pty Ltd v Anderson [2003]*



2. Mapping the territory: how

- Common 'rule': Cost-effectiveness
- Exceptions to the 'rule':
 - 'Life Saving Drugs' program
 - 'Highly Specialised Treatments' or HSTs (>200k/year/patient) (introduced 2020)
- No legal duties of eg fairness
- Patient/public consultation process not enshrined



2. Mapping the territory: how

Judicial guidance?



2. Mapping the territory

Judicial guidance?

Gleeson CJ in *Graham Barclay Oysters Pty Ltd v Ryan* 2002) 211 CLR 540 at [6]:

Decisions as to raising revenue, and setting priorities in the allocation of public funds between competing claims on scarce resources, are essentially political... At the centre of the law of negligence is the concept of reasonableness. When courts are invited to pass judgment on the reasonableness of governmental action or inaction, they may be confronted by issues that are **inappropriate for judicial resolution, and that, in a representative democracy, are ordinarily decided through the political process.**



2. Mapping the territory

Judicial guidance?

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- No merits review
- Limited judicial review - procedural fairness

→ funding decisions are difficult to challenge

→ transparency is the main lever of accountability



3. Law of the ‘public purse’: findings

- Cost effectiveness - rule determined and applied by the Government and advisory committees (and discretion to make exceptions)
- Funding decisions as too political for courts to intervene
- Lack of judicial guidance re funding decisions
- Discretion vs accountability: flexibility, but also fragility





4. Next: how are decisions being made?





Australian Government

Medical Services Advisory Committee

Public Summary Document

Application No. 1623 – Luxturna™ (voretigene neparvovec) for treatment of biallelic RPE-65-mediated Inherited Retinal Dystrophies

Applicant: Novartis Pharmaceuticals Australia Pty Ltd

Date of MSAC consideration: MSAC 79th Meeting, 28-29 July 2020
MSAC 80th Meeting, 26-27 November 2020

Context for decision: MSAC makes its advice in accordance with its Terms of Reference, [visit the MSAC website](#)

1. Purpose of application

Medical Services Advisory Committee (MSAC) Public Summary Document

Application No. 1748 - Review of tisagenlecleucel (Kymriah®) for treatment of acute lymphoblastic leukaemia in paediatric and young adult patients (pALL)

Applicant: Novartis Pharmaceuticals Australia (Novartis)

Date of MSAC consideration: 27 July 2023

Context for decision: MSAC makes its advice in accordance with its Terms of Reference, [visit the MSAC website](#)

1. Purpose of application

Public subsidy for tisagenlecleucel (TIS) to treat paediatric and young adult patients up to 25 years of age with B-cell precursor acute lymphoblastic leukaemia (pALL) that is refractory, in relapse post-transplant, or in second or later relapse, through the National Health Reform Agreements (NHRA) commenced on 31 July 2019. The Department of Health and Aged Care wrote to Novartis Pharmaceuticals (Novartis) in September 2022, providing notice that the review of TIS in pALL had been scheduled for consideration at the July 2023 Medical Services Advisory

Discussion

sara.atinger@sydney.edu.au