Pharmaceutical Benefits Scheme

- The Smoke and Mirrors of Reimbursement -

AMCA
Australian
Medicinal Cannabis Association

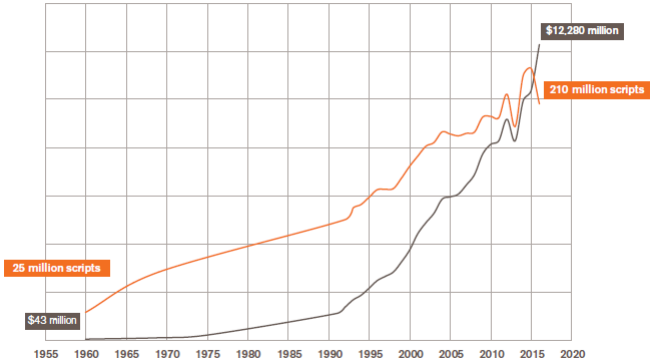
- Reimbursement Interest Group -

Date: 27 July 2021

SYNEVi

Pharmaceutical Benefits Scheme

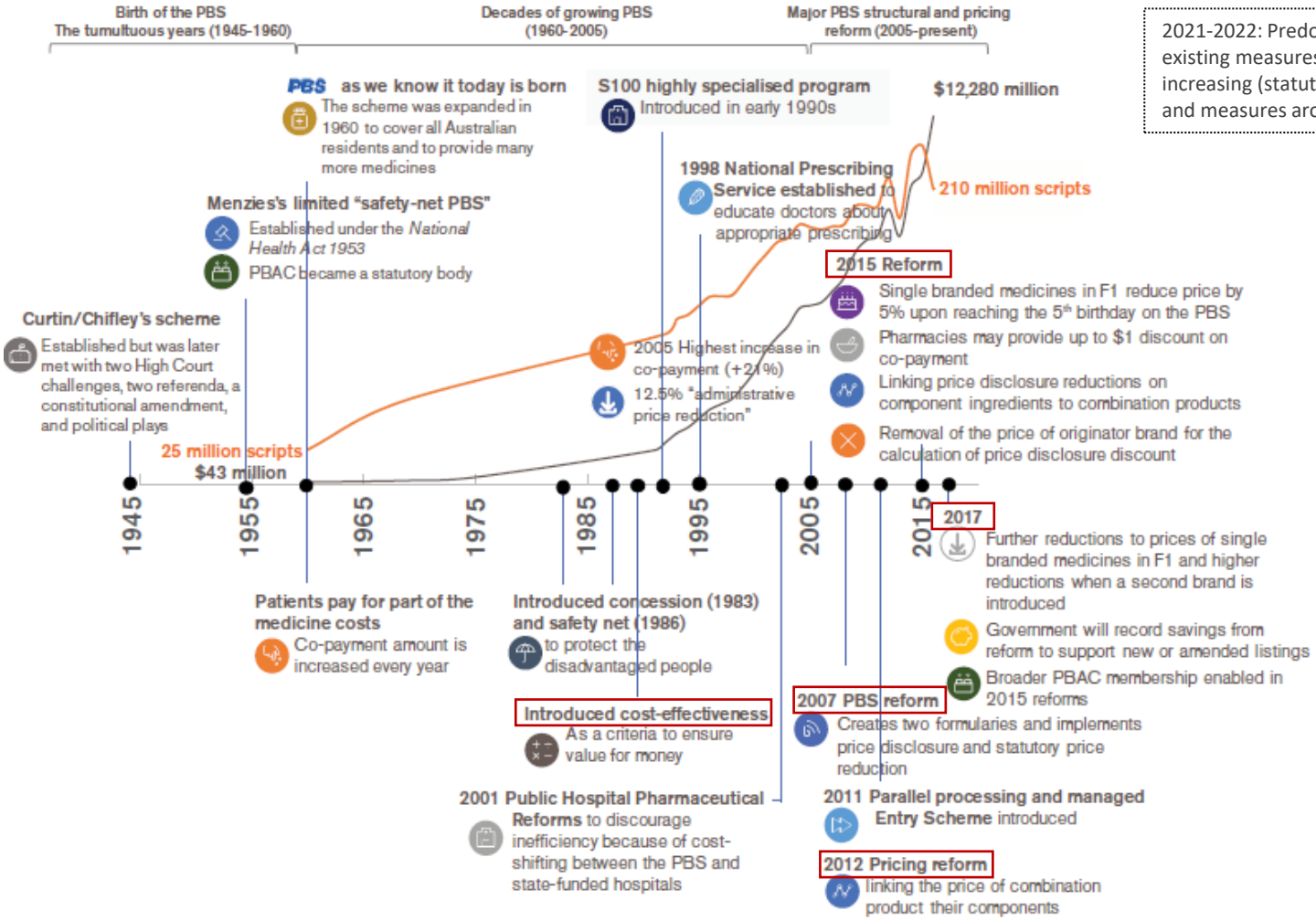
- Pharmaceutical Benefits Scheme (PBS)
  - Established as a limited scheme in 1948
    - Free medicines for pensioners
    - List of 139 ' life-saving and disease preventing medicines for others in the community
  - The 'Golden' years (1960-2005)
    - The PBS has grown substantially in both:
      - Number of medicines that are supplied; and
      - Expenditure/cost to the Commonwealth
    - Reasons for substantial growth include:
      - Medicare creation in the early 1980's
      - Availability of more medicines (e.g., Statins)
      - Introduction of more complex and higher priced medicines
      - Increase in the eligible population
  - In response, a wide range of policy measures were implemented
    - Increasing patient co-payments
    - Introduction of:
      - Concessional category to protect low-income earners and the unemployed
      - PBS Safety net; a maximum patient expenditure threshold
      - Highly Specialised Drug Program (S100)
      - Therapeutic groups
      - Public hospital pharmaceutical reforms
    - De-listing non-essential medicines
    - Printing of the cost of the medicine on the prescribing labels
    - Cost-effectiveness criterion
      - Aim: ensure value for money.
      - A mandatory consideration for the PBAC starting in 1993 and continues to this day



Source: The PBS in Australia: An explainer on system components, February 2018

Pharmaceutical Benefits Scheme

- Pharmaceutical Benefits Scheme (PBS)
  - Policy responses have since become part of the process to ensure a sustainable PBS

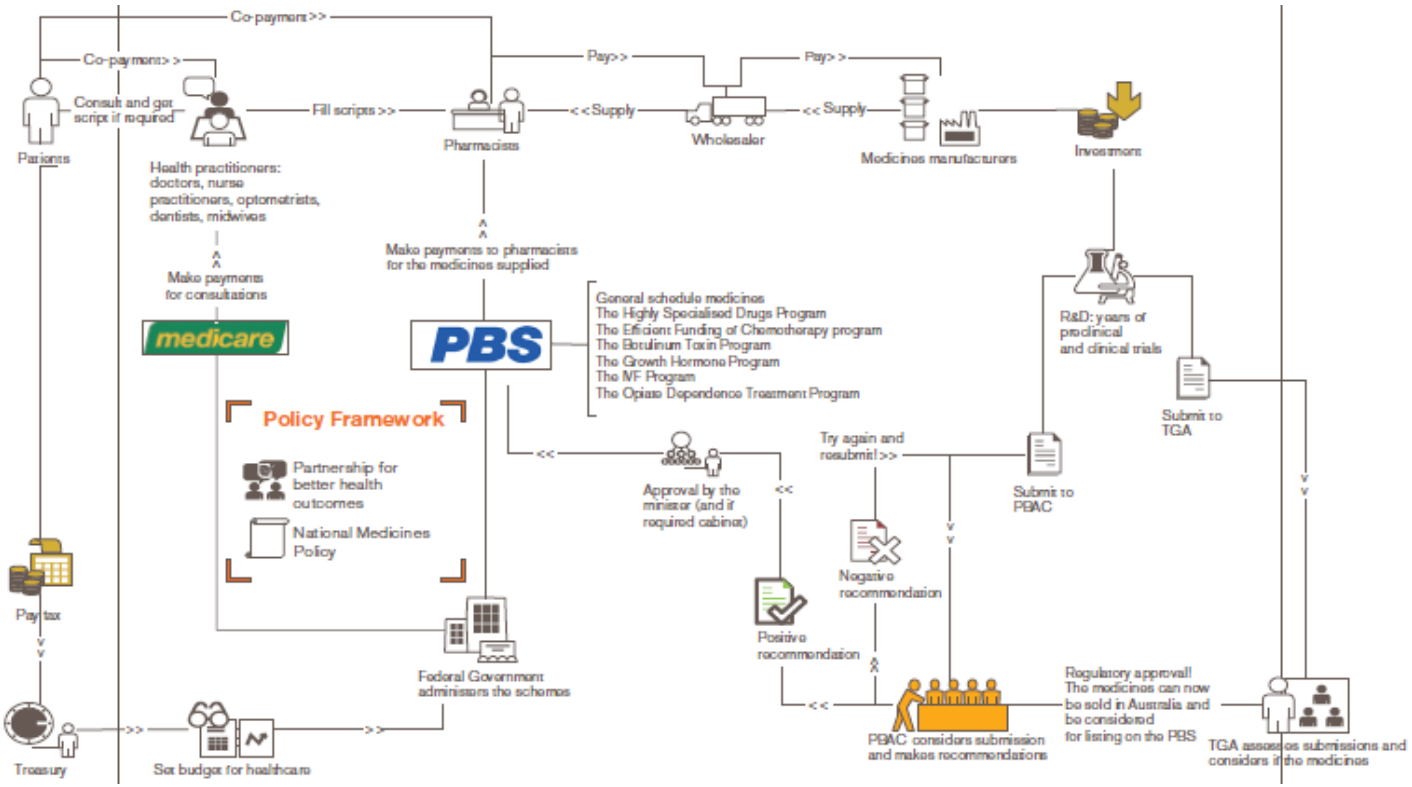


2021-2022: Predominantly variations of existing measures mostly focused on increasing (statutory) price reductions and measures around price disclosure

Source: The PBS in Australia: An explainer on system components, February 2018

Pharmaceutical Benefits Scheme

- Pharmaceutical Benefits Scheme (PBS)
  - Today, there are thousands of medicines listed on the PBS
  - Getting access to a PBS subsidised medicine is easy….
    - Step 1. A health care practitioner (e.g., doctor, dentist, midwives, nurse) writes a prescription
    - Step 2. The patient takes the script to the pharmacy and pays the co-payment (i.e., $41.30 or $6.60)
    - Step 3. Pharmacist hands the medicine to the patient
  - Or is it……



Source: The PBS in Australia: An explainer on system components, February 2018

Pharmaceutical Benefits Scheme

- Pharmaceutical Benefits Scheme (PBS)
  - Why then pursue listing on the PBS?
    - Is it a moral dilemma….ability to pay versus equity?
      - Should a medicine be reserved to those with the ability to pay; or
      - Should access to medicines be the same for patients with equal need irrespective of ability to pay?
    - Or a commercial decision…..
      - ATO statistics: taxable income

	2017–18			2018–19		
	Male	Female	Total	Male	Female	Total
Average taxable income ($)	71,917	49,922	**61,217**	73,218	51,382	**62,549**
Median taxable income ($)	54,252	39,058	**45,882**	55,829	40,547	**47,492**
Average net tax ($)	23,241	14,582	**19,248**	23,365	14,687	**19,344**
Median net tax ($)	14,036	8,699	**11,266**	13,801	8,405	**11,024**

Source: ATO individual statistics, accessed July 2021

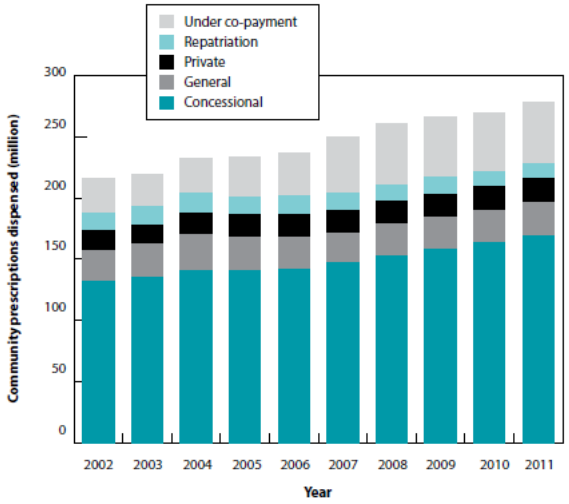
Price versus Volume

PBS:
High volume vs lower price

Private prescription:
Low volume vs higher price

Data from the Australian Statistics on Medicines show that **private prescriptions account for 7-8%** of all prescriptions dispensed in the community pharmacy

Note: no data is collected beyond 2011, but given the evolution in medicines and their cost, if it has changed, this percent could be argued to be less.

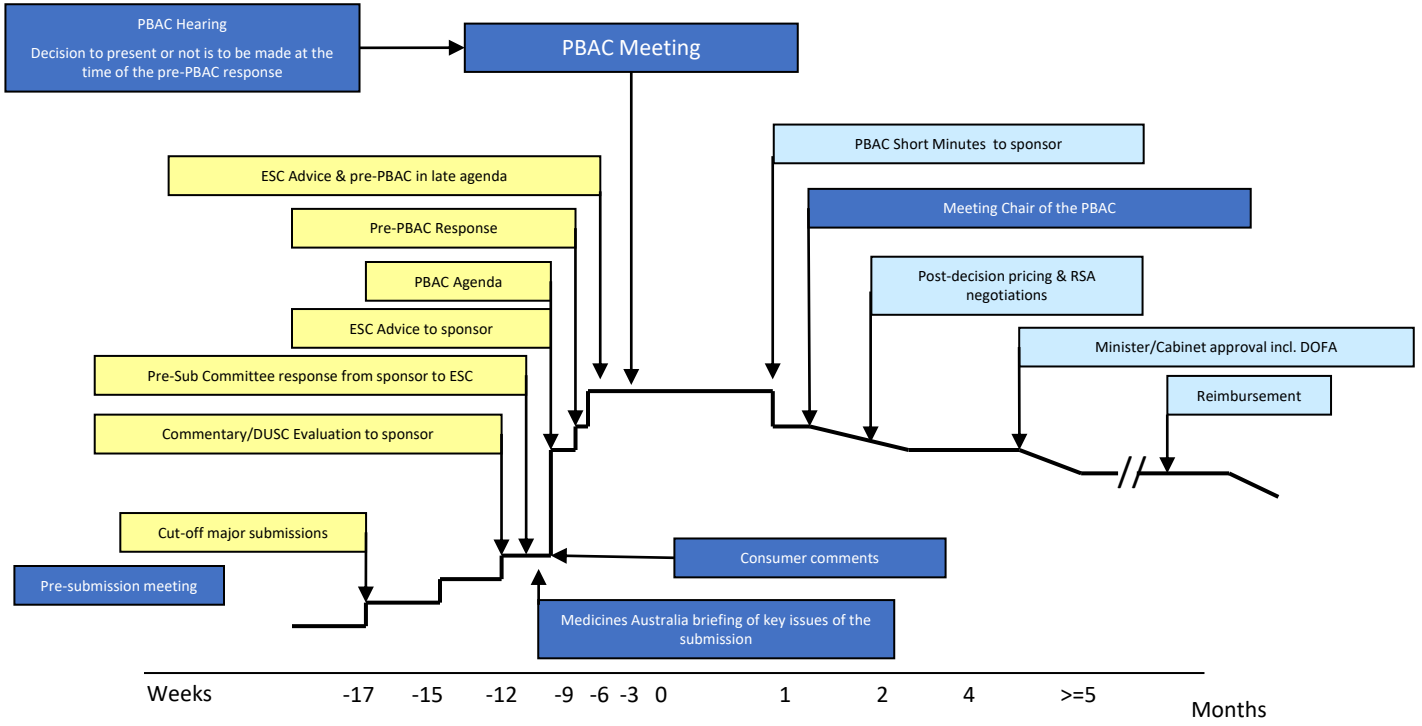


Source: Australian Statistics on Medicines

- The reality is that it is a combination of the above and other factors (e.g., political, patients)

PBAC Submissions

- PBAC submissions
  - The PBAC submission process
    - Some say…….it is complex and lengthy

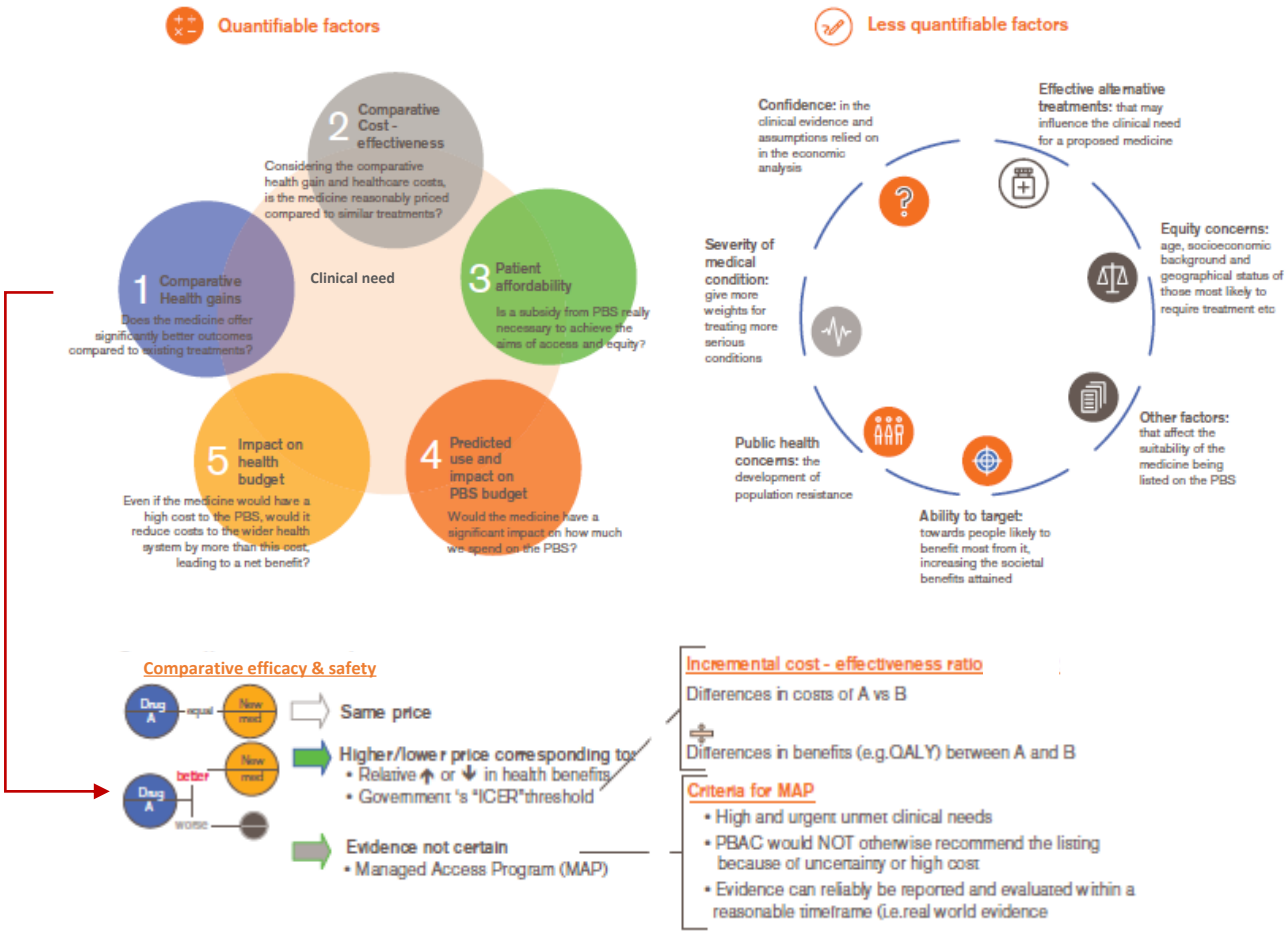


    - ….and costly
      - Category 1: $225,120
      - Category 2: $170,050
      - Category 3: $43,360
      - Category 4: $33,980

Relevant category for most new medicines and/or new indications

PBAC Submissions

- PBAC submissions
  - Making a submission to the PBAC
    - Factors that are considered when assessing an application for reimbursement



Source: The PBS in Australia: An explainer on system components, February 2018

PBAC Submissions

- PBAC submissions
  - Making a submission to the PBAC
    - The actual submission document
      - Prepare in accordance with the Guidelines for submissions to the PBAC

Guidelines for preparing a submission to the Pharmaceutical Benefits Advisory Committee

Version 5.0

September 2016

Australian Government

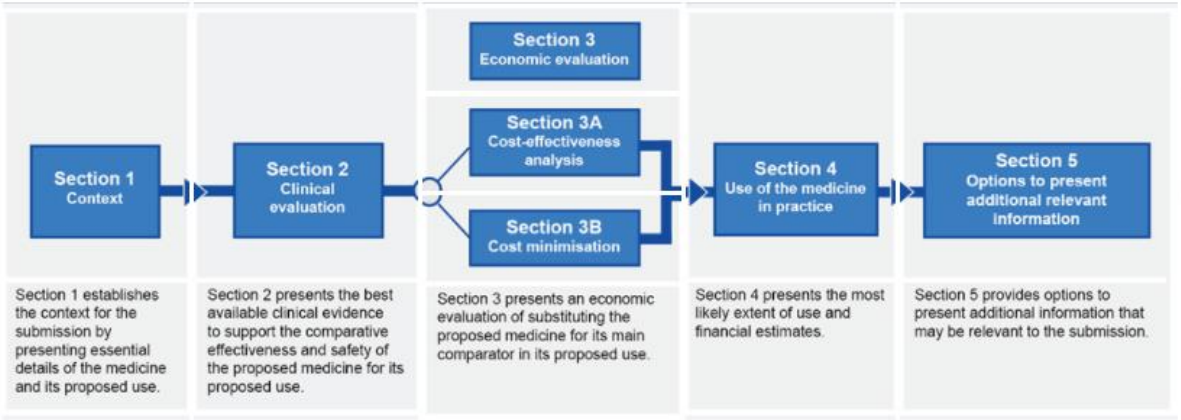
Department of Health

= 205 pages of 'guidance' that is perhaps best interpreted as 'instructions for use'

Option 1 (eventually)

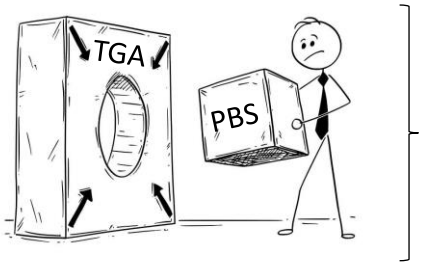
Option 2 (inevitable)

      - Submission document structure is aligned with the factors that are being considered by the PBAC



PBAC Submissions

- PBAC submissions
  - Making a submission to the PBAC
    - Important factors to be aware of….
      - Eligible population: TGA versus PBS
        - The eligible PBS population is the same or smaller than the population approved by the TGA indication
        - Starting point for the PBS population is (almost) always defined based on the in- and exclusion criteria of the clinical trials



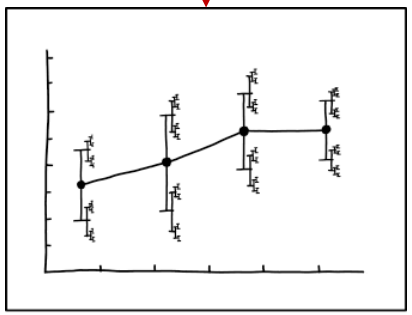
Important to consider PBS reimbursement/environment when making the application for registration to the TGA, especially when it comes to defining the indication to maximise the opportunity, reduce potential complexity of the PBS submission, and maximise (minimise) (mis)alignment.

Always consider what the potential challenges could come back from TGA and asses what, if any, implications this might have on the reimbursement submission

      - Clinical need
        - Simple question ‘what is the clinical need for this medicine’, but sometimes overlooked and not always easy to describe
        - Important question to get right given the relationship between clinical need and willingness to pay (i.e., ICER threshold)
      - Comparator
        - The assessment of efficacy and safety is always comparative; what will my medicine replace in clinical practice
        - Be mindful that the comparator:
          - Is not always a single medicine (i.e., there can be multiple alternative medicines)
          - Can be a sequence of medicines (i.e., comparison of treatment algorithms)
      - Clinical evidence (i.e., comparative efficacy & safety)
        - Perhaps the most important section of the submission, but
        - Success of acceptance of the therapeutic conclusions is largely determined by the quality of the clinical evidence

PBAC Submissions

- PBAC submissions
  - Making a submission to the PBAC
    - Important factors to be aware of….
      - Clinical evidence (i.e., comparative efficacy & safety)
        - Most common challenges with the clinical evidence
          - Clinical trial design
            - Aimed at achieving marketing approval (i.e., TGA registration)
            - Comparator arm not relevant or representative of current clinical practice
            - Dosing in the trial inconsistent with that recommended in the product information
            - Unclear differentiation in dosing regimens
            - In- and exclusion criteria too strict/vague for the result of the study to be applicable to the PBS population
            - Time point at which outcome data are collected
            - Inadequate follow-up
            - Etc.



          - Outcomes
            - Outcome of interest to the PBAC
              - Not collected
              - Not the primary outcome (i.e., not powered to detect a difference)
              - Cannot be translated into meaningful patient relevant outcomes
            - Outcomes in the trials are not routinely or practicable for use in clinical practice
            - Available data for outcomes are immature
            - No consideration of or token inclusion of outcomes that are relevant for the purpose of HTA
              - Quality of life (QoL) performance status instruments (e.g., SF-36) rather than a multi-attribute utility instrument
              - Collect QoL data using a Visual Analogue Scale (VAS)
              - Use of QoL instruments by convenience (e.g., EQ-5D) rather than by design
              - QoL data are collected at a limited number of visit and up until the patient ceases randomised treatment
              - Incorrect statistical analysis of the QoL data
              - Etc.
            - Collection of outcome data that can end up being used against the proposed medicine (e.g., resource use)
            - Etc.

PBAC Submissions

- PBAC submissions
  - Making a submission to the PBAC
    - Important factors to be aware of….
      - Economic evaluation
        - Global cost-effectiveness models are like a pair of jeans
        - Models that are not grounded in the clinical evidence (i.e., Section 2)
        - Clinically implausible assumptions around long-term treatment effect (i.e., beyond the observed trial period)
        - Inadequate consideration of resource use and particularly, the use of medicines in subsequent ‘lines’ of treatment
        - Overly complex models
        - Use of programming (e.g., VBA) to make the model ‘work’
        - Overly optimistic time horizons
        - Etc.

=

One size fits no one

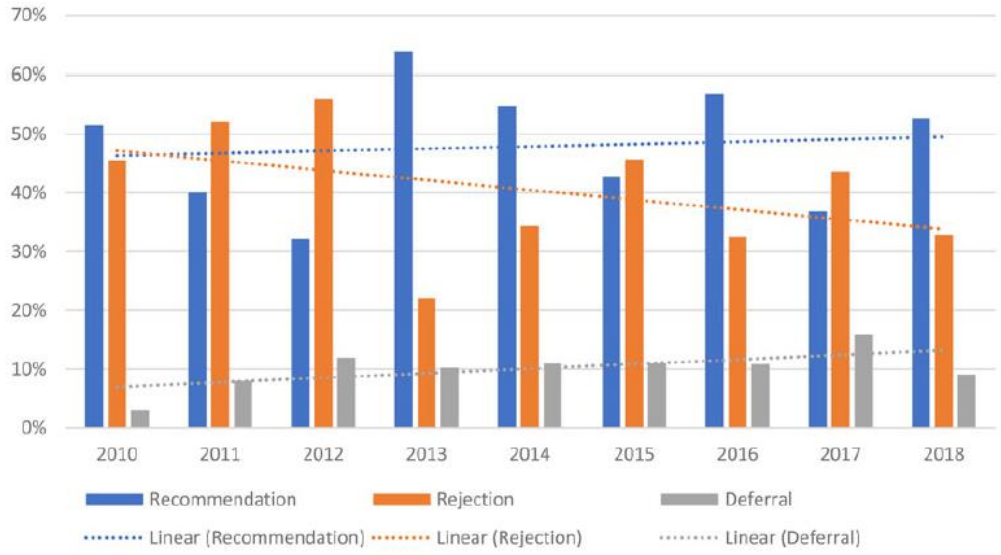
    - Individually these factors might not seem always significant but when viewed together, the PBAC will quickly conclude that…..
      - The place in therapy…
      - The need for the medicine…
      - The magnitude of the benefit…
      - The cost-effectiveness…

    - Is uncertainty managed?
      - Yes, but not necessarily in a manner that favours companies….
        - Reject the application sometimes this is paired ‘guidance’ around what and how issues should be addressed in a new submission
        - Almost always a significant reduction in price is required
        - Risk-sharing arrangements are imposed with most of, if not all, the ‘sharing’ done by the company

PBAC Submissions

- PBAC submissions
  - Making a submission to the PBAC
    - How then do I approach reimbursement….
      - Plan and consider HTA alongside marketing authorisation….early in clinical development program
      - Design suitable clinical trials that collect appropriate and relevant outcomes
      - Be mindful that cost-effectiveness models are bespoke pieces of art
      - Objectively assess the entirety of the clinical evidence, and don't only focus on the bits you like

    - Ultimately, it's a process without guarantees
      - PBAC outcomes (recommended, rejected, deferred)



Source: Wonder & Lybrand., 2020

PBAC Submissions

- PBAC submissions
  - Making a submission to the PBAC
    - Ultimately, it's a process without guarantees
      - Number of submission to achieve a recommendation

Category	Submission attempts (*n*)	Recommendations (*n*)	Average number of submission attempts to obtain a recommendation
All	875	514	1.70
CEA	405	172	2.35
CCA[a]	5	4	1.25
CMA	369	268	1.38
CA[b]	14	11	1.27
Not required	68	50	1.36
Not available[c]	7	4	1.75
Unknown[d]	7	5	1.40

[a]Cost consequences analysis.
[b]Cost analysis.
[c]An economic evaluation was not included in the submission but should have been.
[d]Unknown because there is no PSD.

    - If approached properly and with a realistic understanding of the process, evidence, environment and price expectations it is simply….

And with patience, persistence and determination, all the pins fall for most medicines (i.e., recommended for PBS listing)

Source: Wonder & Lybrand., 2020

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