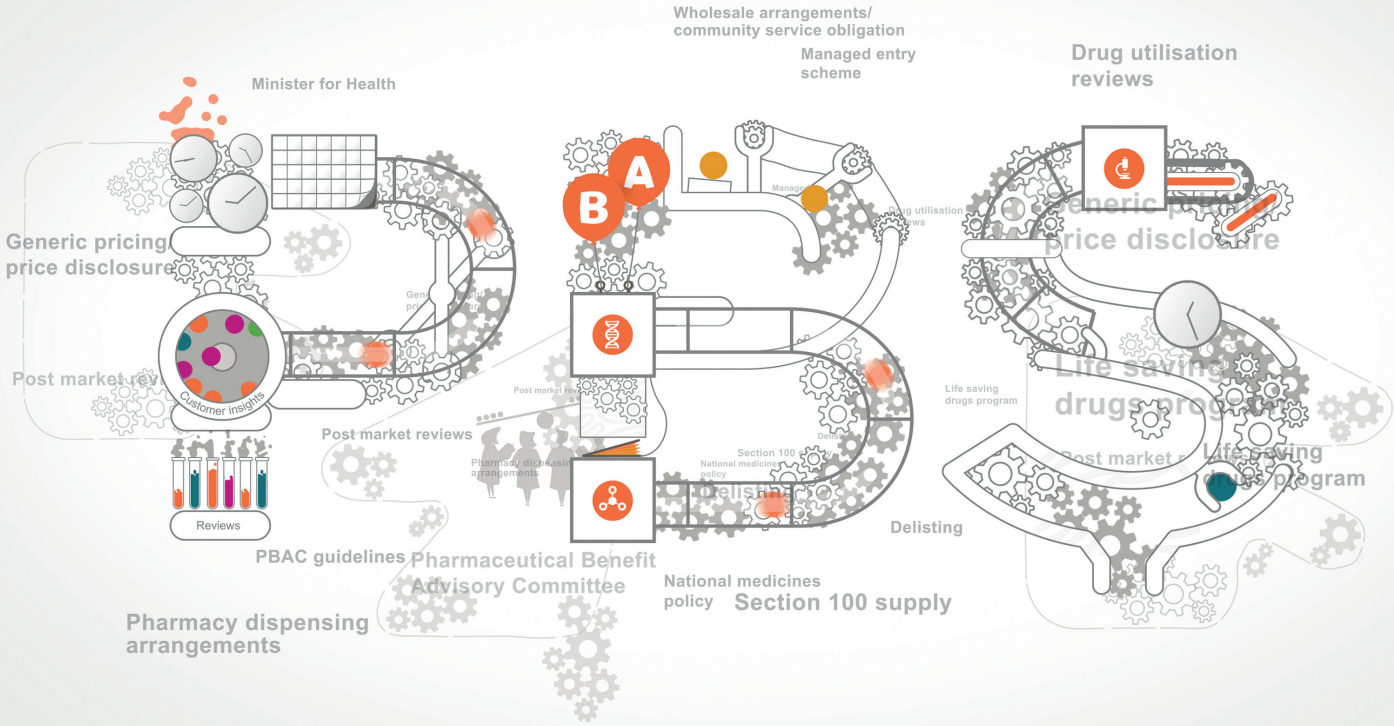

The Pharmaceutical Benefits Scheme in Australia

An explainer on system components
February 2018

This document was prepared by GlaxoSmithKline Australia Pty Ltd and ViiV Healthcare Pty Ltd with the assistance of Deloitte Access Economics Pty Ltd

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Foreword

Australia's commitment to universal access to healthcare is a source of pride for many Australians, including our employees at GlaxoSmithKline Australia (GSK) and ViiV Healthcare (ViiV). But how do we define universal access to healthcare?

A healthcare system based on the philosophy of universal access is one that removes financial barriers for patients. In practice, this means all Australians can access the latest and most appropriate medical, pharmaceutical, biological and vaccines advances when necessary.

The Australian Federal Government provides universal access to healthcare, such as medical services, via Medicare and to medicines through the Pharmaceutical Benefits Scheme (PBS). Without the PBS, access to medicines could be difficult for many Australians. For example, due to the large investment required for research and development, some medicines may cost thousands of dollars to treat a single patient. But when listed on the PBS, the same treatment is subsidised and is available through a modest patient co-payment.

Even though the PBS is accessible to all Australian citizens, many only become involved with the Scheme when a medicine is prescribed to them, or they require access to a medicine that is not yet available through the Scheme. If a patient or their carer seeks to understand the rationale for why this is so, it is our experience that many will be overwhelmed by the complexity of the laws, policies and processes that determine this.

In response to this, GSK and ViiV have collaborated to "*pull back the curtain*" on the PBS, to help explain some of its complexity and to foster a common understanding of the system. In consultation with the community and other stakeholders, we have developed a short video and report that explains how the PBS operates and highlights stakeholder perspectives on the tensions and considerations that exist within it. For example, this document outlines perspectives on the challenge of balancing individual patient preferences in a national scheme and how the scheme could evolve in the future.

As the Australian population continues to grow and age, and the advancements in treatments enable greater benefit and health outcomes, the demand on our healthcare systems will increase. Therefore, continued investment and evolution will be required. For the PBS to remain a hallmark of Australia's universal healthcare system, we need to ensure it evolves in a way that enables ongoing timely patient access to advancements in medicines and vaccines, incentivises continued medical innovation whilst also remaining fiscally responsible.

The aim of this paper and accompanying video is to provide an engaging, constructive and informative resource to aid understanding of a complex and often emotive policy issue. We hope it enables an ongoing informed discussion on the Australian healthcare system and how the PBS will evolve to continue to provide universal access to innovative medicines and vaccines when people need them.

GSK and ViiV welcome your feedback and encourage you to get involved and have your say. It is your PBS.

Anne Belcher
General Manager
GSK Australia

About GSK

GSK is a science-led global healthcare company, committed to researching, developing and providing access to innovative pharmaceuticals, vaccines and consumer healthcare products in more than 100 countries around the world. Our goal is to enable people to do more, feel better and live longer.

Michael Grant
Country Manager
ViiV Healthcare

About ViiV Healthcare

ViiV Healthcare is an independent, science-led, global specialist HIV company with a broad portfolio of antiretroviral medicines, an industry leading pipeline, and several first of their kind programmes to improve access to medicines and support novel on the ground community initiatives.

Acronyms

Acronym	Description
ACPM	Advisory Committee on Prescription Medicines
ATAGI	Australian Technical Advisory Group on Immunisation
ATC	Anatomical Therapeutic Chemical classification
CDL	Combination Drugs List
DHS	Department of Human Services
DVA	Department of Veterans' Affairs
DUSC	Drug Utilisation Sub Committee of the Pharmaceutical Benefits Advisory Committee
EMA	European Medicines Agency
ESC	Economics Sub Committee of the Pharmaceutical Benefits Advisory Committee
F1	Formulary 1
F2	Formulary 2
FDA	Food and Drugs Administration
GDP	Gross Domestic Product
HSD	Section 100 Highly Specialised Drugs Program
ICER	Incremental cost effectiveness ratio
NIP	National Immunisation Program
PBAC	Pharmaceutical Benefits Advisory Committee
PBS	Pharmaceutical Benefits Scheme
RSA	Risk share agreement
TGA	Therapeutic Goods Administration

Glossary

Term	Description
Advisory Committee on Prescription Medicines (ACPM)	An expert body that provides advice and makes recommendations to the TGA, on whether manufacturers could sell goods for therapeutic purposes in Australia.
Authority required medicines	Prescribed medicines that require prior approval from the Department of Human Services of Department of Veterans' Affairs.
Comparator	A medicine's current alternative therapy or therapies in Australia, most likely to be replaced in clinical practice if that medicine is listed.
Comparator price erosion	The reduction in the linked price of a medicine due to a reduction in the price of its comparator.
Cost-effectiveness analysis	Comparing the cost or price of a medicine with its effectiveness relative to that of its comparator. In principle, more effective medicines will be priced higher, and less effective medicines will be priced lower.
Cost-minimisation analysis	Reviewing the cost or price of a medicine relative to a comparator that has similar effectiveness, with the goal of achieving lower cost for similar health outcomes.
Drug Utilisation Sub Committee	The Drug Utilisation Sub Committee (DUSC) of the Pharmaceutical Benefits Advisory Committee (PBAC) assesses estimates on projected usage and financial cost for medicines submitted to the PBAC for PBS listing.
Economic modelling	Estimating the expenditure on a medicine that would result from accepting a positive listing, to determine the effect on the PBS budget.
Economics Sub Committee	The Economics Sub Committee (ESC) of the Pharmaceutical Benefits Advisory Committee (PBAC) assesses clinical and economic evaluations of medicines submitted to the PBAC for PBS listing, and advises PBAC on the technical aspects of these evaluations.
European Medicines Agency	A regulatory body responsible for the scientific evaluation, supervision and safety monitoring of medicines in the European Union.
Formulary	A list of medicines subsidised under the PBS. There are two main formularies within the PBS, Formulary 1 for single-brand medicines and Formulary 2 for multi-brand medicines.
Inferior	A medicine is inferior if it is not as effective as its comparator.
Managed Access Programme	A scheme that allows listing of medicines that meet an urgent unmet clinical need despite less sufficient clinical evidence or uncertainty over cost-effectiveness

Term	Description
Medicare	Publicly funded universal health care system in Australia.
Medicines Australia	An organisation that represents the discovery-driven pharmaceutical industry in Australia.
National Health Act 1953	The Act gives effect to the powers of the PBAC and governs the PBS listing, supply and funding arrangements.
National Immunisation Program (NIP)	Is an established collaborative program involving the Australian Government and the state and territory governments, which aims to increase national immunisation coverage rates by funding essential vaccines for eligible infants, children, adolescents and adults.
Non-inferior	A medicine is non-inferior if its effectiveness is equal to or better than that of its comparator.
Observational studies	A study design whereby there is no randomisation used to determine who receives a treatment. These studies can over or under-state effectiveness if the people who receive a treatment are systematically different to those who do not.
Parallel processing	A process whereby a manufacturer can apply for PBS listing while their medicine is still being assessed by the Therapeutic Goods Administration (TGA).
Patient co-payment	The amount paid by a consumer to access a medicine listed on the PBS. As of 2017, this is $38.80 per script, or $6.30 for concession cardholders.
Prescription	An instruction written by a medical practitioner that authorizes a patient to be issued with a medicine or treatment. Often referred to informally as a Script.
Price disclosure	A policy that requires medicine manufacturers to disclose to the Government the amount they charge to pharmacists for medicines.
Quality Adjusted Life Year (QALY)	Is a well accepted measure of disease burden, including both the quality and quantity of life lived. It is used in economic evaluations to assess the value for money of medical interventions.
Randomised controlled trial	A study design whereby participants who receive and do not receive a medicine (the treatment and control groups) are selected randomly to help remove any biases in the measurement of a medicine's effectiveness.
Restricted medicines	Medicines that can only be prescribed if a patient's condition meets stated requirements.
Rule of rescue	A principle that favours listing of medicines for severe conditions affecting a small number of patients where no existing treatment exists, and the medicine offers a significant increase in chances of patient survival.

Term	Description
Statutory price reduction	Any instance where the Government compulsorily reduces the price paid for a medicine to manufacturers.
Superior	A medicine is superior to its comparator if it is more effective.
Unrestricted medicines	Unrestricted medicines may be prescribed at the prescriber's discretion.
US Food and Drug Administration	The organisation in the US responsible for determining the safety and efficacy of medicines, similar to Australia's Therapeutic Goods Administration (TGA).

1 Pulling back the PBS curtain

In Australia, we're lucky to have access to government funded health care.

One of the pillars of our healthcare system is the Pharmaceutical Benefits Scheme. The PBS commenced in the late 1940s with a limited number of medicines. Today, it subsidises thousands of medicines used in community settings, hospitals and specialist clinics. The aim of the PBS is to ensure that all Australians – regardless of how much or little they earn, or where they are in society – can have access to high quality and affordable medicines when they need them.

Over the years, successive Australian governments have implemented a suite of reforms to the PBS so the system can respond to the changing healthcare needs of Australians while remaining financially sustainable. These reforms have focused on new ways of assessing and listing medicines, and have often delivered savings and value for money to the Government and ordinary Australians, which is great. But they have also created a complex system with numerous sets of rules and processes. To name a few, these rules and processes include:

- various assessment criteria for deciding whether a medicine should be listed on the PBS;
- changes to the listing processes to allow for faster access to medicines;
- legal requirements for the manufacturers of medicines to disclose to the Government how much they charge and how many packs of medicines they sell;
- linking prices between medicines;
- reducing prices based on how long medicines have been on the PBS; and
- many post listing reviews, including reviews to see if medicines are used as they were initially intended.

But making policies in a complex system like the PBS can cut both ways. Changes to one part of the system may bring benefits, but cause unanticipated impacts in another. Indeed, with complexity in rules and processes, the PBS system presents many points of tension among stakeholders. How should a national scheme like the PBS weigh individual preferences against the needs of the whole population? How should the Government maintain financial responsibility when so many Australians expect fast access to new medicines that are often expensive? While technical analyses and complex decision-making processes are seemingly necessary, do they lose sight of the human dimension of providing access to affordable medicines to people with specific medical needs when they need it – the fundamental promise of the PBS?

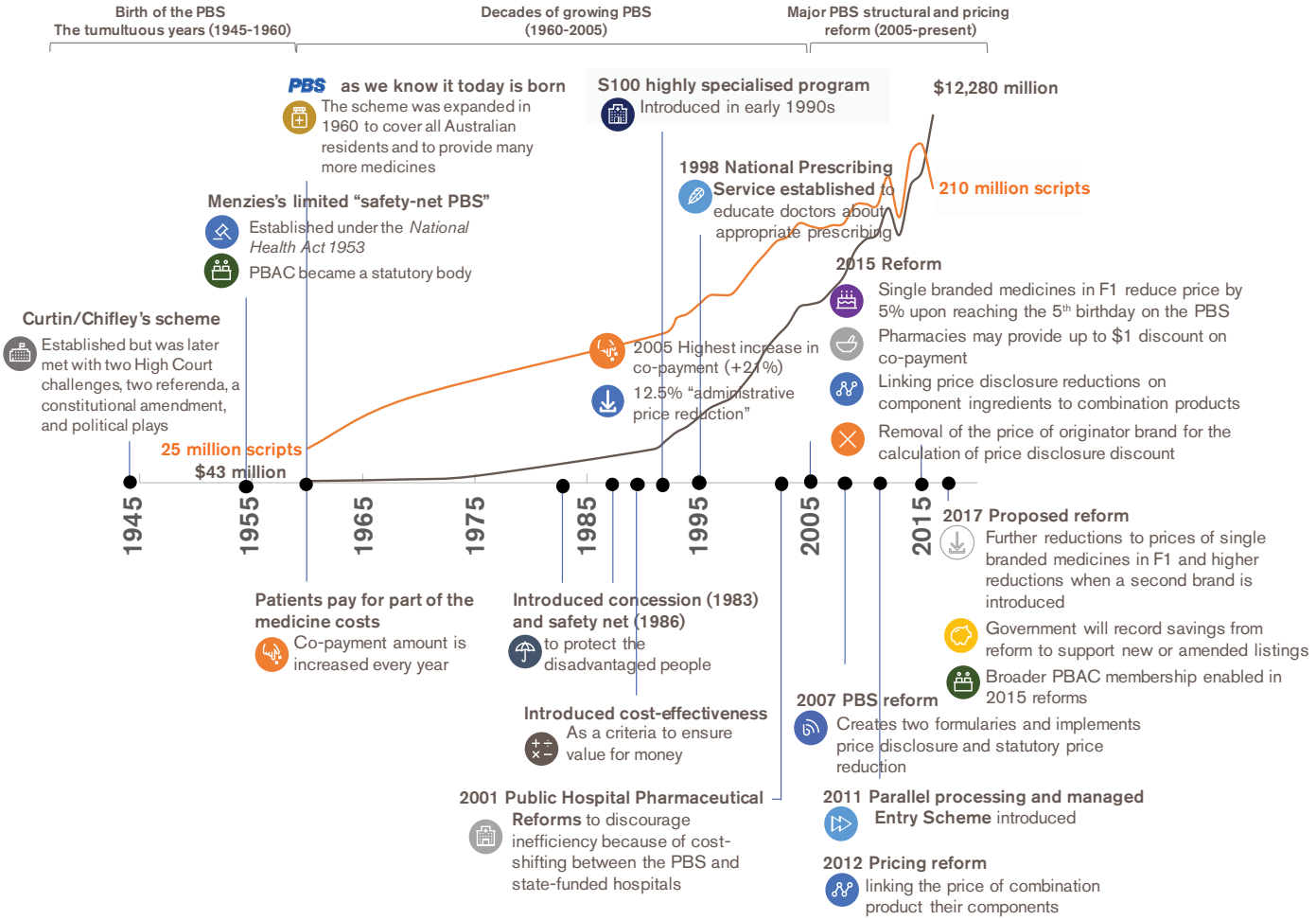
Patient advocacy, peak bodies and community groups have provided feedback that the PBS system is difficult to fully understand because the rules and processes are technical and complicated. This means they sometimes have difficulties in holding a meaningful policy discussion, let alone understanding how the current system and any recent reforms may have an impact on patient access to PBS medicines.

GSK and ViiV Healthcare (ViiV) believe it is important for all stakeholders to fully understand the mechanics of the PBS. Only with a better understanding of how the system works can stakeholders share their views and play an important role in maintaining a robust and sustainable PBS – one that will serve Australia now and well into the future.

It is to this end that GSK and ViiV have jointly developed this document to 'pull back the PBS curtain', to be read alongside the video presented at [au.GSK.com], by explaining PBS mechanisms in a clear and accessible way for interested stakeholders. It also highlights some of the tensions that exist within this complex system.

This document aims to introduce some of the core components of the PBS to the extent possible while remaining concise. For this reason, it does not describe some aspects of the PBS in detail e.g. how patients access chemotherapy, how the Government pays wholesalers and pharmacists for supplying medicines, or how patients gain access to diagnostic testing to make sure that the medicines are appropriately used.

2 A brief history of the PBS



PBS in 2017 in a nutshell

How is PBS managed?

- Run by the Federal Government
- Laws are set in the National Health Act 1953
- Pharmaceutical Benefits Advisory Committee (PBAC) recommends to the Minister which medicines should go onto the PBS . The members of PBAC include doctors, health professionals, health economists and consumer representatives.
- Parallel processing and managed Entry Scheme were introduced to speed up patient access to new medicines

What medicines are covered by PBS?

- Medicines used in community settings (e.g. blood pressure medications)
- S100 Highly Specialised Drugs Program for medicines used at specialist settings (e.g. cancer treatment)
- Medicines for specialised programs (e.g. growth hormones, medicines for IVF)
- In some states, medicines supplied to patients on discharge

What are some of the policies to control costs and ensure value?

- Patient contribution called "co-payment"
- Patient pay less if they have concession or when their annual spending has reached the safety net
- PBAC must consider cost-effectiveness of medicines
- Manufacturers need to disclose their selling prices and volumes (price disclosure)
- Prices of combination products linked to their components, including reductions due to price disclosure
- Single branded medicines in F1 reduce price by 5% upon reaching the 5th birthday on the PBS
- Pharmacies may provide up to $1 discount on co-payment
- Removal of the price of originator brand for the calculation of price disclosure discount
- Government will record savings from reform to support new or amended listings

2.1 Birth of the PBS: the tumultuous years of 1945-1960

The birth of PBS is the result of many hard-fought battles.

The PBS was first envisioned during the Second World War (WWII) to provide medicines to returned servicemen.[a] During the war, the Curtin Government (1939-1945) saw a need to provide access to innovative lifesaving antibiotics – sulphonamides, streptomycin and penicillin. As part of its broader agenda on creating a tax-funded national welfare scheme, the Curtin Government wanted to ensure that war veterans and all Australians could afford not only antibiotics but also a more comprehensive list of essential medicines. With this vision in mind, the wartime Curtin/Chifley Government enacted the *Pharmaceutical Benefits Act 1944* after overcoming numerous political barriers and general ideological opposition to a welfare state. Under Curtin/Chifley's scheme, Australian residents were eligible to receive free prescription medicines from community pharmacies, so long as those medicines were listed in a "formulary" drawn up by an expert committee.

Following Curtin's death in 1945, the Chifley Government (1945-1949) took on the baton for implementing the PBS, as well as battling through ongoing opposition to the scheme. In particular, the British Medical Association (BMA) in Australia bitterly opposed the PBS, claiming that "socialised medicine" was the first step on a slippery slope towards socialism. It is more likely that the BMA was simply in opposition to any government intervention that would affect the working conditions and pricing power of doctors, and their income by extension.[1] Unsurprisingly, the first six years of the PBS turned out to be a failure in practice: there were very few medicines prescribed under the scheme because of a boycott by more than 98% of Australian doctors. By 1950, the PBS had fought for its survival against two High Court challenges, two referenda, a constitutional amendment, and an immense level of political and ideological power play.

> *In 1944 a new agent, streptomycin, was refined from a soil fungus; in time, it would revolutionise tuberculosis treatment. The press ran many heartbreaking stories of people unable to afford the new "miracle drug".*
>
> *As the public demanded access to this new era of medicine, the three preconditions for universal health care aligned for the first time: new ideas on the function of the state, the medical tools to save millions of lives and a government with the political will to act.*
>
> *Goddard 2014[1]*

It was only under the following Menzies Government (1949-1966) that the 'new' PBS, as we know it today, gradually evolved over the decade leading to March 1960.[1] In the 1950s, Menzies' "safety-net PBS" only included a small number of "expensive and life-saving" medicines for pensioners – the elderly, invalids, widows or servicemen and service women. During this time, the Pharmaceutical Benefits Advisory Committee, or PBAC, also became an independent statutory body under the *National Health Act 1953* to maintain the formulary – the Schedule of Pharmaceutical Benefits. By 1960, the limited list no longer met the community expectations of the time. Following the passage of the *National Health Act 1959,* the PBS list finally became more comprehensive and universal as initially envisioned. The Government also introduced co-payments, where patients were required to pay five shillings for each prescription. This is equivalent to $7.13 in 2016 dollars.[b]

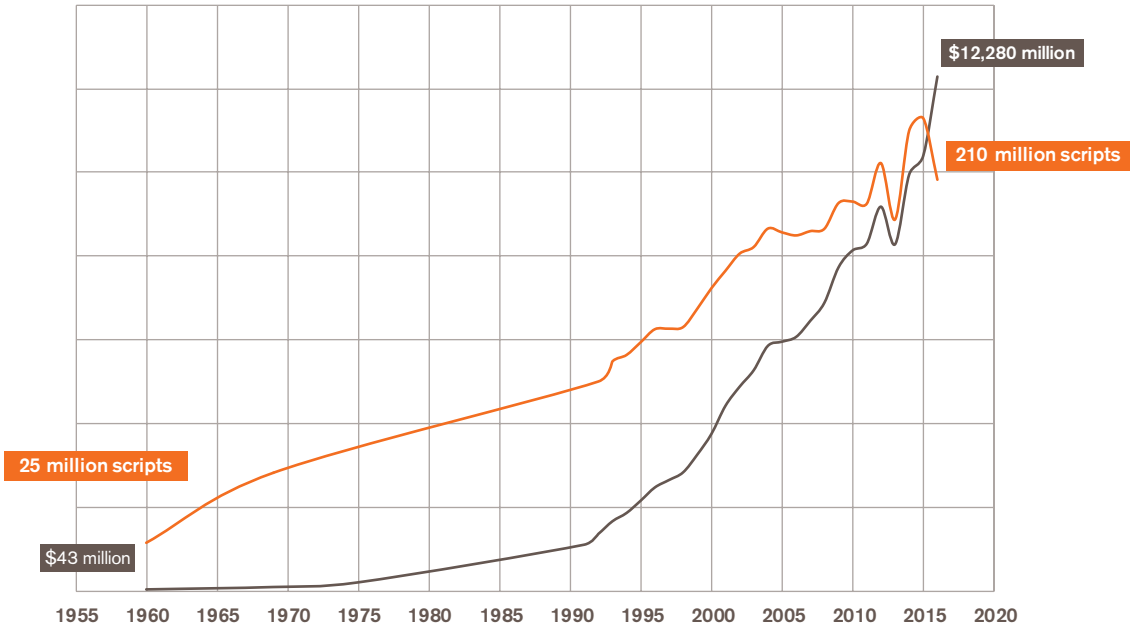
2.2 The growing decades of PBS: 1960-2005

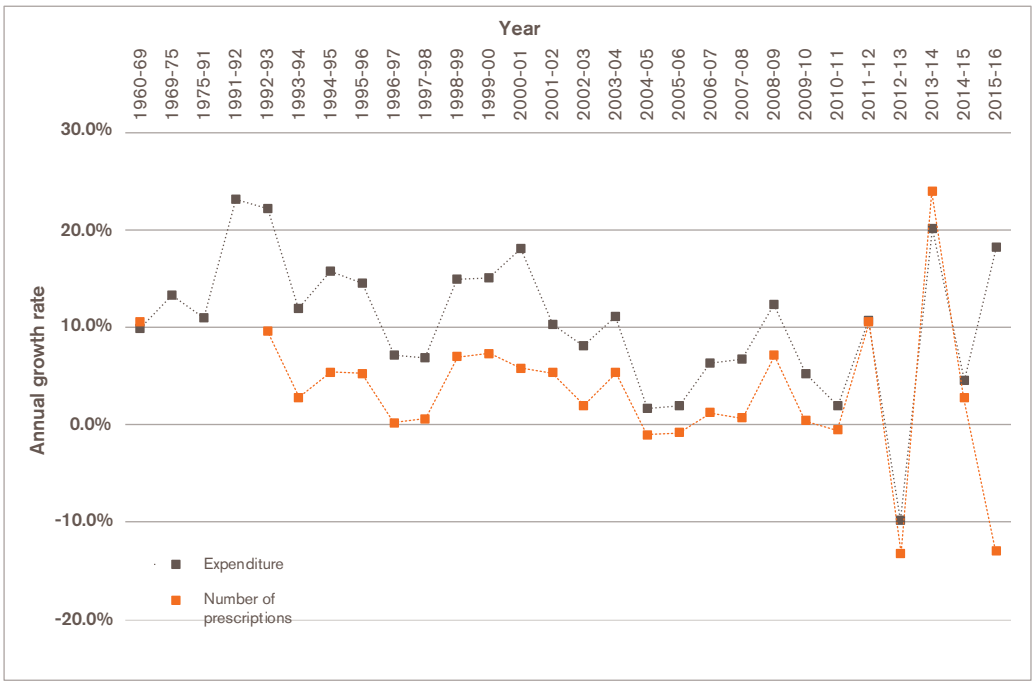
In the four decades from its commencement, the PBS experienced considerable growth, both in the number of medicines supplied and in government expenditure. Official data indicate that expenditure grew in the double digits between 1960 and 2004 (see Figure 2.1) and the expenditure was rising much faster than the number of scripts. There are a number of reasons for the explosive growth over the period, including:

[a]A similar scheme was in place in 1919 for war veterans from the First World War and the Boer War.
[b]Reserve Bank of Australia's Pre-Decimal Inflation Calculator: www.rba.gov.au/calculator/annualPreDecimal.html

- the creation of Medicare in the early 1980s;
- the availability of more medicines for more treatments (e.g. the introduction of statins for reducing blood cholesterol in the 1990s, after evidence showing a reduction in heart events such as heart attacks, chest pain and deaths due to heart disease[2]);
- a shift towards new, more complex and often higher priced medicines;
- an increase in the proportion of the population eligible for concession cards and an ageing population; and
- over-prescribing and use of medicines in an unintended patient population.[3]

Figure 2.1: PBS prescriptions and expenditure, 1960-2016, by numbers and growth rate





Source: Briggs 2003 and Medicare Statistics (n.b cost and growth in expenditure outlined in Figure 2.1 is prior to company rebates to government for high cost medicines and risk sharing arrangements. In reality the cost of the PBS has been lower due to company rebates. See section 3.5 on page 17 for more information).

In response to the growing costs and changing healthcare needs of Australia, successive governments have implemented a range of policy changes. These policy changes aim to ensure value for money, budget control and financial protection for disadvantaged people with a lesser ability to pay for medicines. These policies include:

- **From 1960:** Gradually increasing the amount of money patients need to pay for part of the cost of a medicine. This "co-payment" is to prevent people from getting medicines unnecessarily just because they are free;
- **1983:** Introducing a concessional category so that the disadvantaged, such as low-income earners and the unemployed, pay less for their medicines;
- **1986**: Introducing a 'safety net' so that singles and families who require a lot of medicine only need to pay up to a set amount each year in co-payments, or pay less for each script once their spending reaches a certain threshold;
- **1987-1993**: Introducing a new "cost-effectiveness" criterion to ensure value for money. From 1993, the PBAC was required to consider whether the benefits of a given medicine were high enough to justify the price requested by a manufacturer before listing.
- **Early 1990s**: Introducing the highly specialised drugs (HSD) program to supply medicines via hospital out patient departments under section 100 of the National Health Act 1953 – now called "S100 drugs";
- **1998**: Setting a base price for medicines that are not chemically identical but have similar clinical effects (i.e. a therapeutic group). Patients now pay for the difference between the higher priced medicine and the base price in addition to their co-payment;
- **1998**: Establishing the National Prescribing Service to inform and educate doctors about appropriate prescribing;
- **1996-97, 1997-98, 2000-2001**: Delisting 'non-essential' medicines for the first time, such as topical antifungals, some anti-inflammatory medicines, and nasal sprays;
- **2001:** Implementing a package of Public Hospital Pharmaceutical Reforms to improve patient care and discourage inefficiency/inconvenience because of cost-shifting between the PBS and state-funded hospitals;
- **2003**: Printing the full cost of PBS medicines on medicine labels, accompanied by a controversial $24 million government-commissioned advertising campaign – "the prescription for a healthy PBS" – to educate patients about the cost of medicines.

2.3 Major PBS structural and pricing reforms from 2005

The story of major PBS reforms after 2005 starts with the maiden Intergenerational Report in 2002. In this report, the Australian Treasury highlighted that the PBS was one of the fastest growing areas of government spending in the decade to 2000-2010. It also suggested that this growth was only partially because there are more Australians or Australians are getting older. Treasury's prediction warned that "in 40 years' time, the PBS could account for 3.4% of GDP, making it the largest part of the Government's spending on health".[4]

In response, the report triggered several reforms that have changed how the PBS sets medicine prices in an effort to ensure the sustainability of the system. First, in 2005, the Government imposed the **highest increase in patient co-payments** and safety-net thresholds in the history of PBS.[5] The Government said this was necessary "to help restore the balance between Government and patient contributions to the PBS".[6] The Government also introduced "administrative price reduction" where a manufacturer wanting to list a new brand (i.e generic version) of a PBS medicine would need to offer at least a 12.5% price discount. This would later become a requirement by law, to be known as "**statutory price reduction**".

[c] F2 at the time was also split into F2A and F2T so that the Government could apply different pricing rules.

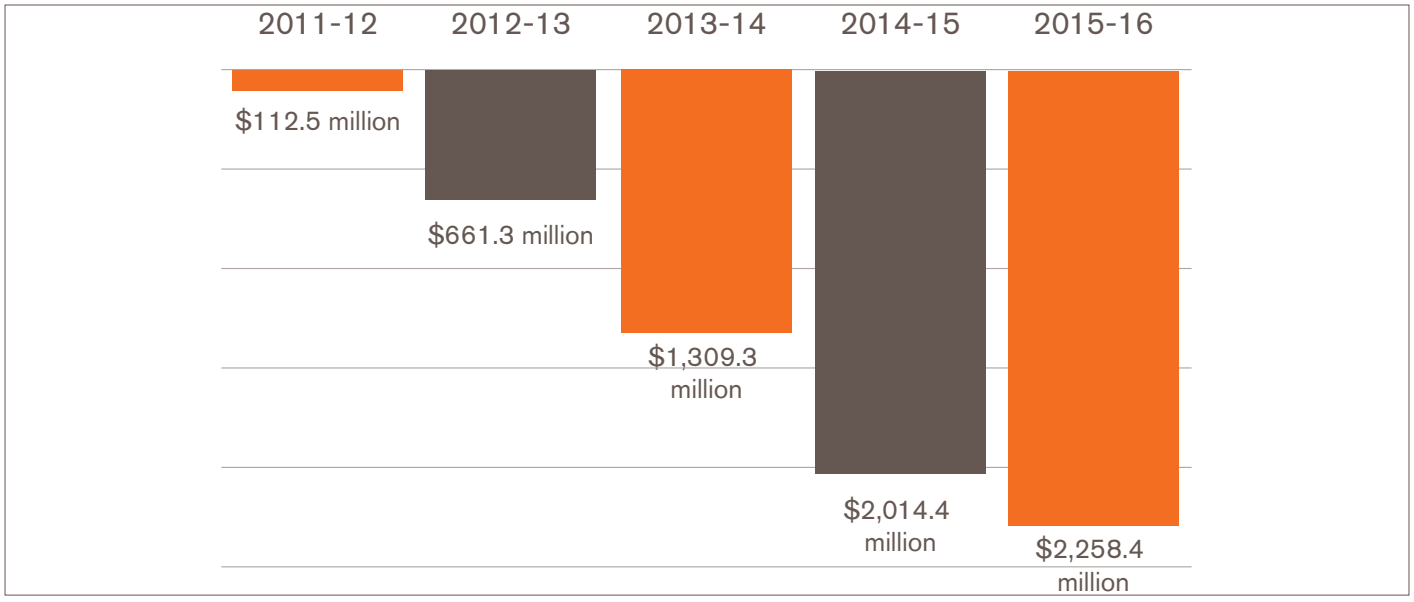
Then, in 2007, a package of reforms to restructure the PBS pricing arrangement took effect. Many elements of this package of reforms form the basic architecture of the PBS, as we know it today. Among other changes, the reform created **two PBS formularies**. Formulary One (F1) consists of medicines with only one brand. F1 medicines are typically on-patent and the first medicine of its type to be on the PBS. Formulary Two (F2)[c] consists of PBS medicines with multiple brands. F2 medicines are typically off-patent generic medicines subject to competition. The purpose of splitting the formulary was to apply separate sets of pricing rules to each formulary. Based on the separate pricing rules, the Government can extract savings from the competition of off-patent medicines with multiple brands; at the same time, the Government can also make sure that the prices of patented single-brand F1 medicines are set according to their value through cost-effectiveness evaluation, so that the system can continue to encourage and incentivise innovative therapies. In explaining the rationale for splitting the formulary in the Parliament before introducing the law, the then Minister for Health and Ageing noted that:

> "the prices of many drugs on the PBS are linked, with no distinction between drugs with single brands where there may be no suitable alternatives for patients, and those with multiple brands that are operating in a competitive market. It has been difficult for the government to pay competitive prices for multiple brand drugs as these prices could flow on to other essential drugs resulting in their withdrawal."[7]

When the law came into effect, the Government made it compulsory for the manufacturer to lower the price of medicines in the F2 Formulary via a statutory price reduction prescribed in the National Health Act 1953. But there was no price cut for medicines in F1.

Furthermore, to capture the benefits of competition for medicines with multiple brands in F2, the Government also introduced a pricing arrangement called "**price disclosure**" (Section 4.3 explains this in more detail). Before the introduction of this measure, the Government had been paying more than the market price for medicines with multiple brands, because manufacturers sold medicines to pharmacists for less than the PBS price. By making manufacturers disclose their selling prices and volumes, the Government could adjust the PBS price according to the market price and reap the benefits of competition. Price disclosure was initially applicable to a smaller number of medicines in F2 and in 2010 it became a requirement for all medicines in F2. To date, the price disclosure arrangements have resulted in price reductions of many medicines in F2, generating billions in savings to the Government and taxpayers (Figure 2.2).

Figure 2.2: Savings from price disclosure measures, 2011-2016



Source: RSM, Financial analysis of pharmacy regulations and remuneration arrangements, March 2017. Cited in: Australian Government Department of Health 2017, Review of Pharmacy Remuneration and Regulation Interim Report. Canberra: Commonwealth of Australia.

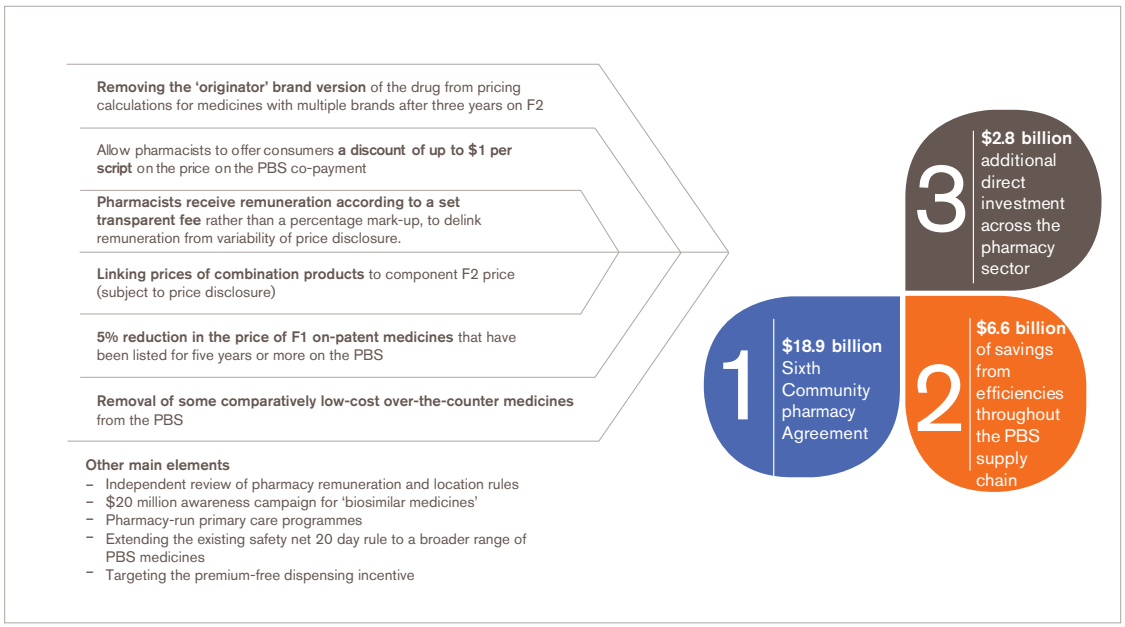
In 2011, the Government worked with the medicines industry and other stakeholders to implement two measures in an attempt to **speed up the process** to list a medicine on the PBS. The first measure was **'parallel processing'**. Parallel processing allows the PBAC to consider a submission to list a new medicine on the PBS while the Therapeutic Goods Administration (TGA) is considering if the medicine is of good quality, safe and efficacious. However, PBS listing still requires the medicine to be first approved by the TGA. The second measure is the creation of the "**Managed Entry Scheme**" or MES, now called the "**Managed Access Programme**", whereby the PBAC may recommend a listing of a medicine "at a price justified by the existing evidence, pending submission of more conclusive evidence of cost-effectiveness to support listing of the drug at a higher price".[8] In effect, the Managed Access Program provides earlier patient access to promising medicines meeting certain criteria, with an agreement between the sponsor and the government to collect further data that supports its use, value and price.

One example where these measures had worked well to speed up patient access to new medicine is the case of pembrolizumab – a medicine to treat melanoma that has spread or when surgery could not completely remove the tumour. The manufacturer worked with the Department of Health collaboratively and put the medicine through parallel processing and MES. This resulted in the listing of pembrolizumab on the PBS on 1 September 2015, only 4.5 months after TGA granted approval. Under the MES, the PBAC reviewed further evidence in November 2016 ensuring that doctors and patients had used pembrolizumab according to the best practice and that the cost and cost-effectiveness of pembrolizumab were acceptable. Pembrolizumab continues to be available through the PBS following this review.

From 2012, in consultation with the stakeholders, the Government has also implemented a range of **pricing measures on medicines that have multiple components,** or "combination products". First, the Government implemented a policy to link the prices of "single brand" combination products to the prices of their components.

Later, the Government in 2015 implemented more reforms as part of the *Pharmaceutical Benefits Scheme Access and Sustainability Package*. This reform package was included under the Sixth Community Pharmacy Agreement – a five-yearly agreement since 1990 between the Pharmacy Guild of Australia and the Government to set an agreement *usually* about how retail pharmacies supply PBS medicines (i.e. how much pharmacists get paid for dispensing and so on[d]). Figure 2.3 lists the main components of the reform package.

Figure 2.3: Reform of policies under the 2015 Pharmaceutical Benefits Scheme Access and Sustainability Package



[d]Although the Community Pharmacy Agreements have been an important part of the PBS policy, it is not the intention of this report to cover this topic. Please refer to http://6cpa.com.au/about-6cpa/ for further information if you would like to learn more about it.

In 2017, in collaboration with the innovative medicines industry, and in consultation with other stakeholders, the Government announced further pricing reform as part of the proposed 2017-18 budget. The *Strategic Agreement* with Medicines Australia includes some pricing measures (e.g. further price reductions for medicines in F1), measures to improve the listing process (e.g. focusing resources on the more complex PBAC submissions) and measures to promote patient access to medicines (e.g. recording savings accrued from policy changes to support investment in new listings of medicines). The agreement commits to policy and pricing stability over five years. The proposed changes would require approval from the Federal Parliament before they come into effect.

Years of Government reforms have delivered savings and value for money to Government and the Australian community. As shown in Figure 2.1, changes in expenditure from 2004-05 onwards are mostly below 10% and in line with the changes in script volumes. The only exception is in 2015-16 when the Government listed highly efficacious but high cost medicines for hepatitis C. Indeed, access to high cost medicines via the PBS, particularly high cost cancer medicines, have been subject to much debate over recent years. It should be noted however that the cost and growth in expenditure outlined in Figure 2.1 is prior to company rebates to government for high cost medicines and risk sharing arrangements. In reality the cost of the PBS has been lower due to company rebates. See section 3.5 on page 17 for more information.

This section has described some of the major reforms for the PBS, but it is intentionally brief and by no means comprehensive. There are in fact a suite of other complex policies and history of iterative reforms that affect, for example, how patients access chemotherapy, how the Government pays wholesalers and pharmacists for supplying medicines, how patients gain access to diagnostic testing to make sure that the medicines are appropriately used, and so on. These reforms are the result of combined effort between the Government, industry, doctors, pharmacists and patients to continue making the PBS system sustainable and effective in meeting the needs of Australians.

2.4 The PBS today

As you can see, the PBS we have today has come a long way from the limited scheme introduced by Curtin and Chifley. Ordinary Australians, governments, healthcare professionals and educators, and the medicines industry have all put a tremendous amount of effort over the years to make the PBS happen, and to make the PBS better – often in partnership with one another.

Today, the PBS operates within the policy framework of Australia's National Medicines Policy (NMP), that has been in place since 2000. **Stakeholders across society should continue to be involved in the national discussion about overall principles and mechanisms to help ensure that all Australians can enjoy better health from having access to good medicines and using them wisely.**

The NMP states four goals to collaboratively achieve:
- **timely access** to the medicines that Australians need, at a cost individuals and the community can afford;
- **medicines meeting appropriate standards** of quality, safety and efficacy;
- **quality use of medicines;** and
- **maintaining a responsible and viable medicines industry.**

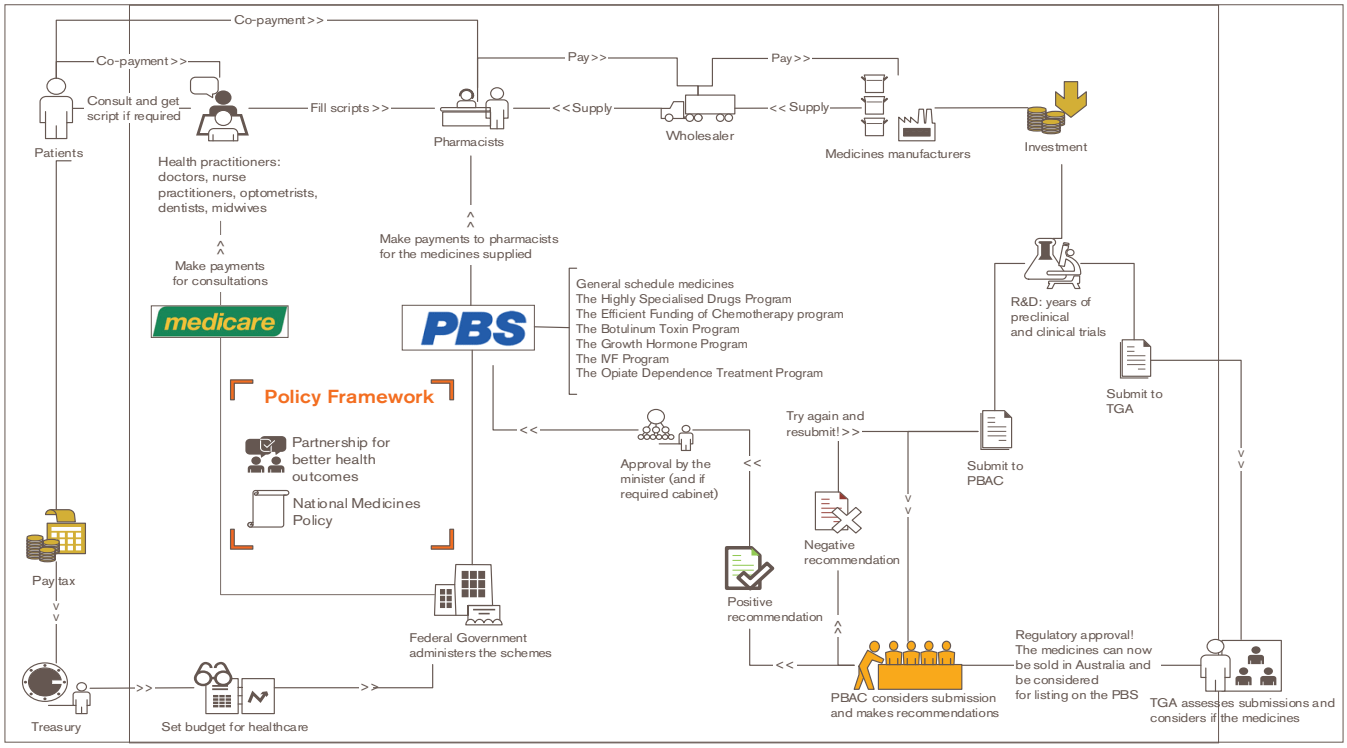
Today, the PBS provides good access to subsidised medicines. Most people know that health practitioners – doctors, dentists, optometrists, midwives and nurse practitioners – may write a script for medicines, which allows patients to get prescribed medicines from an approved pharmacist, simply by paying the co-payment (Figure 2.4, p.10).

[e]The process of research and development involves many different experiments in the laboratory and phases of clinical trials. These are all essential for ensuring that medicines are safe and efficacious before it can be widely used.

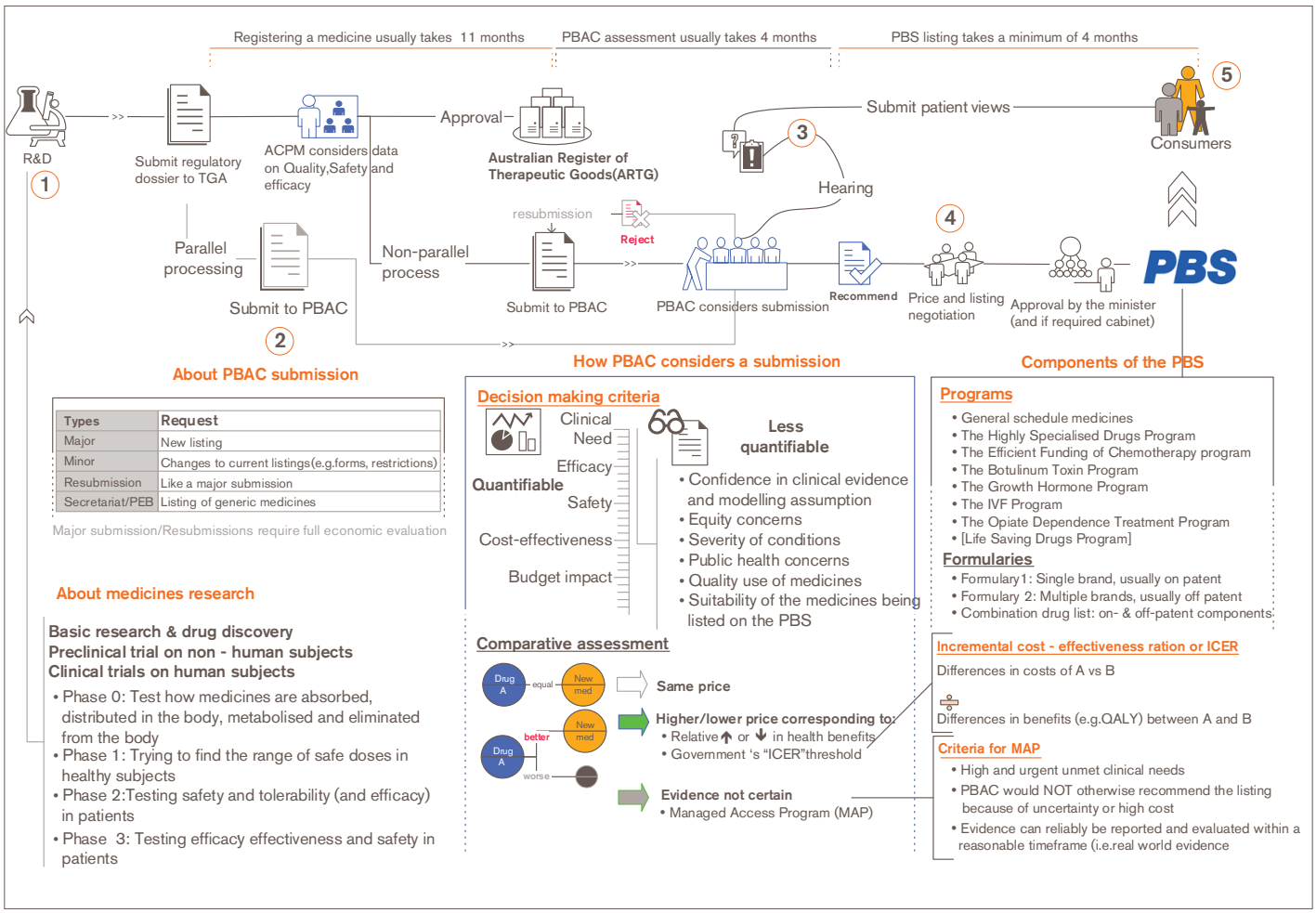
But there's a bit more to the PBS than meets the eye. There's a long and complicated process to get medicines out of the laboratory, and into the hands of patients in need. In order to get a medicine onto the PBS in the first place, there is a long road of compiling clinical evidence[e] and economic evidence so the PBAC can decide whether the medicine should be on the PBS. Second, once a medicine is already on the PBS, there are myriad different rules governing how the prices of listed medicines can change, and how the prices of different medicines tie to one another. Furthermore, as noted in Chapter 1, the complexity of the PBS system also presents many points of tension among stakeholders because of their competing interests and needs. The complexity also generates a range of expected and unexpected consequences because these rules work in most but not all situations. This means that continuing to refine PBS policy is important so that the PBS can continue to meet its purpose and fundamental principles.

In the next chapters, we will outline the main components of the PBS listing and pricing processes, and cut through their complexities.

Figure 2.4: An overview of how patients gain access PBS medicines and how the PBS system works



3 Listing medicines on the PBS



How consumer or consumer groups can get involved

1. Ask your healthcare provider about clinical trail that may be appropriate for you
2. Support preparation of PBAC submissions by letting the manufactures know about your experience and axpectation of the PBS for providing access to medicine
3. Provide your view to PBAC on new medicines listing at PBS.gov.au
4. Monitor PBS reforms in parliment and tell policymakers what you think at APH.gov.au
5. Learn more about the system and stay connected with consumer, healthcare and medicines peak bodies

3.1 Prior to submission to the PBAC: seeking regulatory approval

Before manufacturers can apply for listing a medicine on the PBS, they must first seek approval from the Therapeutic Goods Administration, or TGA. The TGA is a body set up by law to enforce the regulations in the *Therapeutic Goods Act 1989*; accordingly people refer to it as a "regulatory body". It has similar functions to the Food and Drug Administration (FDA) in the United States and the European Medicines Agency (EMA). It is responsible for assessing and monitoring the quality, safety and efficacy of goods sold in Australia for therapeutic purposes. These range from vitamins and sunscreens to over-the-counter and prescription medicines, vaccines, blood products and surgical implants.

For new medicines, the TGA, through an expert body called the Advisory Committee on Prescription Medicines (ACPM) (or the Advisory Committee on Vaccines for new vaccines), investigates information

submitted in a voluminous document called a 'dossier' by the manufacturer or sponsor. In this document, the TGA carefully considers the chemistry of the medicines, manufacturing processes and the results from years of clinical trials undertaken by the manufacturers and their research collaborators (e.g. universities). The TGA's role is to ensure that the medicines Australian consumers buy can be trusted as good quality and safe. For prescription medicines and some OTC medicines, the TGA also carefully examines the efficacy data to make sure that a medicine can do what it claims to do.

If all goes smoothly with the assessment, the medicine will be recorded on the Australian Register for Therapeutic Goods or ARTG approximately **eleven months** from the date of TGA submission. This means that the medicine has obtained "marketing authorisation" and the manufacturer can sell the medicine in Australia, of course, according to the requirements in the regulations. At this point, the TGA-approved medicine could be considered for listing on the PBS, but patients would need to pay full price until it is listed on and subsidised by the PBS.

You may want to note:

- TGA must first approve a medicine before it can be sold in Australia or be listed on the PBS.
- Patients will most often need to pay full price for a TGA-approved medicine until it is subsidised by Government through the PBS.
- Manufacturers can apply for PBS listing of medicines at a similar time as when they apply for TGA approval. This is called 'Parallel processing' and it may get a medicine onto the market and PBS faster.

In some cases, manufacturers can apply for "parallel processing". This is when a manufacturer applies for PBS listing at a similar time as when they apply for TGA approval. If all goes according to plan, this means that the manufacturer can expedite patient access, rather than waiting for TGA approval before applying to the PBS. Of course, PBS listing still requires the TGA first granting 'marketing authorisation' through listing on the ARTG.

3.2 Submission to the PBAC: seeking reimbursement

Chapter 2 explains that the PBAC has been the independent expert body set up since 1953 under the *National Health Act 1953*. Its members are a group of doctors, health professionals, health economists and consumer representatives appointed by the Minister for Health. As at 2017, the PBAC meets three times a year to consider PBS listings. When necessary, the PBAC also convenes ad-hoc meetings throughout the year to discuss PBS-related matters and in some cases, to discuss PBS listings. The Minister for Health and the PBAC are the gatekeepers to the PBS listing process because a new medicine cannot be listed on the PBS unless this committee recommends its listing and the Minister for Health accepts this recommendation (or in some cases, the full Cabinet, for expensive new listings expected to cost over $20 million in any one year).

To do this, the PBAC receives and considers submissions seeking listing of medicines on the PBS. These submissions are usually from manufacturers because the manufacturers have most of the required data from their years of extensive clinical trials. Furthermore, the government charges for evaluating, pricing and listing medicines, vaccines and other products or services on the PBS – a process known as 'cost recovery' – which can be expensive and therefore may not be affordable for many organisations. Having said that, the PBAC can consider submissions from medical bodies, health professionals, private individuals and their representatives, so long as they can provide the data to support the request and meet the costs. An example is the joint submission by Australasian Society for HIV Medicine (ASHM), National Association of People with HIV Australia, Australian Federation of AIDS Organisations in 2013 to request removal of restriction imposed on HIV medicine (anti-retro-viral therapy) so that more people living with HIV could have greater choice about their treatment.

There are four types of submission for listing medicines on the PBS according to the type of request: (1) **Major submission**; (2) **Minor submission**; (3) **Committee secretariat submission**; and (4) **Submission for new brand of an existing pharmaceutical item**. Major submissions request listing of new medicines or vaccines, or listing of a new condition or 'indication' for a medicine that has already been listed on the PBS for a different indication. Major submissions always require an economic

model to support any claim of value for money. The other three types of submissions generally relate to changes to the existing listings that do not materially change the use and value for money of the medicine (e.g. listing of a new form, new strength or a 'home brand' medicine proven to be equivalent to an existing PBS medicines). Economic modelling is normally not required in these latter three types of submissions, and the PBAC may not be involved. This is because the listing requested is relatively straightforward and does not impact, or has minimal impact, on the value for money or budget of the PBS.

In 2017, the Australian Government and Medicines Australia – the body that represents the 'discovery-driven' pharmaceutical industry in Australia – announced a *Strategic Agreement*. This agreement states, among other things, a commitment to focus resources on the more complex PBAC submissions and to simplify processes and adjust the costs associated with less complex PBAC submissions. Under this agreement, the PBAC may also consider changing how frequently it meets. All these proposals may result in changes to the listing processes. So, watch this space.

3.3 How the PBAC considers a submission: comparing, judging and balancing

Suppose that the TGA has now approved a new medicine because the submission from the manufacturer shows that it is effective, safe and of high quality. The PBAC still requires some additional requirements to be met before it decides to recommend that the Minister for Health list that medicine on the PBS. These additional requirements involve comparing the new medicine with medicine(s) or treatment(s) currently available to treat the same condition in people with the same medical condition that the new medicine claims to treat. This is so that the PBAC can consider how much better or worse the new medicine is in changing the health outcomes (e.g. prolonging life, improving symptoms, reducing side effects etc) compared to what the current treatment offers.

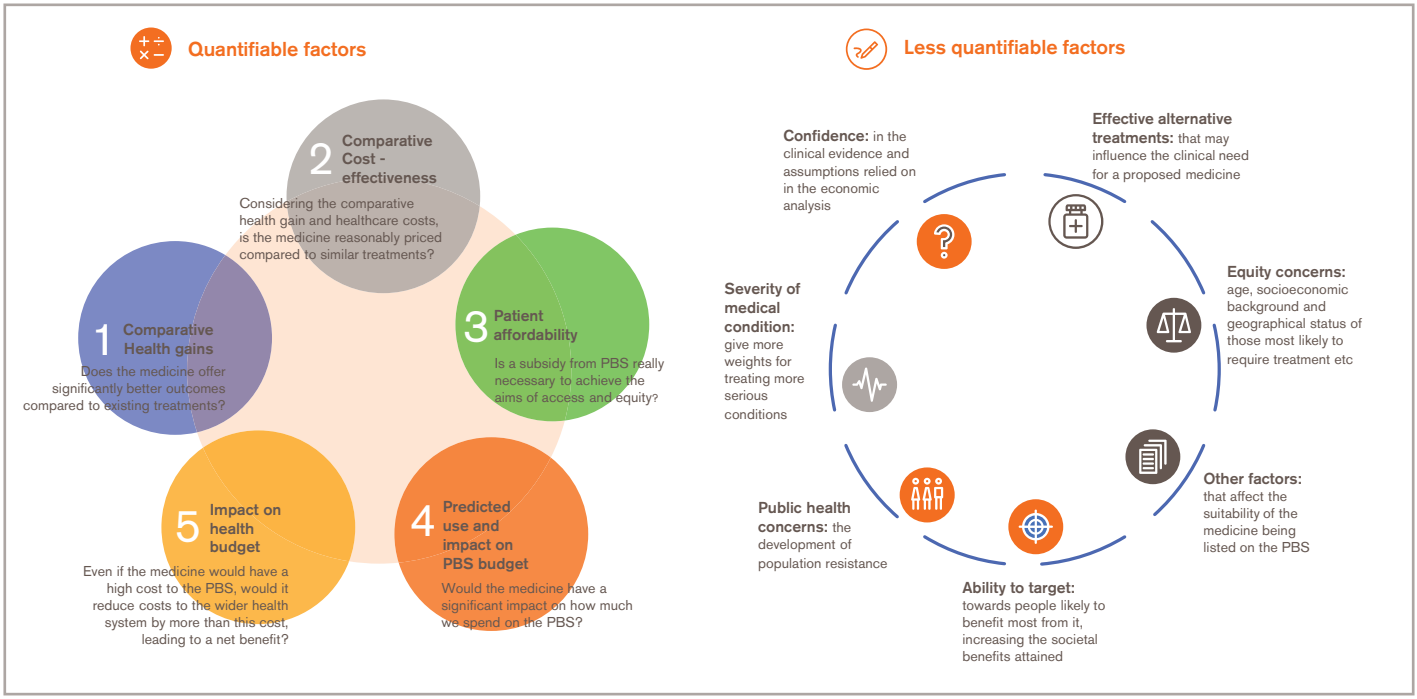
Because there is a limited amount of money in the Australian health budget, not every potential cure or advancement in treatment can be subsidised by the Government through the PBS. This means that the PBAC can only approve a medicine if it considers it will offer value for money. For this reason, the PBAC is required by law to make sure that a medicine must not just be effective and safe, but also "cost-effective" and can maintain the financial health of the PBS.

In this context, when the PBAC is deliberating on whether to list a certain medicine, it follows several guiding principles. These include **five quantitative factors** and a range of **less quantifiable factors** (Figure 3.1).[9] These factors mean that the PBAC relies on technical analyses and a great deal of judgment on what the Australian community expects and values in order to come to an informed decision. The process uses the best available information at the time of the submission, expertise of the committee and input from the community, but it is certainly not a completely exact science.

Many medicines listed on the PBS are only for a corresponding health condition or a particular group of patients. So, a submission to the PBAC needs to specify the condition (e.g. cancer in a particular part of the body) and the patient characteristics (e.g. age and whether a certain genetic makeup is present) for which the new medicine would be used. Accordingly, the PBAC's deliberation is only for this condition and group of patients. Once listed, the medicine may appear multiple times on the PBS for a set of approved conditions and patients. These are called "**restricted medicines"** and some of these medicines require approval[f] (i.e. "**Authority required**"). The purpose of these restrictions is to ensure that the use of the medicines is appropriate (e.g. for appropriate patients authorised by qualified doctors with specialist knowledge). It is also to control 'leakage' and the costs from inappropriate use (e.g. where the medicines or the type of patients have not been shown to the PBAC as sufficiently clinically or cost-effective). This is why sometimes we read about patients not able to access a particular medicine through the PBS, even though the medicine is on the PBS for another condition. For example, in the article "Cancer patient pays $5,000 every two weeks for treatment costing others just $6.20"[10], the patient had a rare cancer of the nerve cell that releases a chemical messenger. She

was not eligible to receive a PBS medicine listed for melanoma, even though some doctors considered the medicine as her last option to extend life. Of course, there are many **unrestricted medicines** and prescribers can write a script for these medicines at their discretion, using their clinical judgement about appropriateness.

Figure 3.1: Factors considered by the PBAC when considering a submission to list a new medicine[g]



3.4 Information presented in a major submission

Going through the information presented in a major submission can help explain what 'evidence' the PBAC is looking for. Overall, there are five sections in a major submission: context, clinical evaluation, economic evaluation, use of medicines in practice and other relevant information. These are explained below and for simplicity, the explanation uses a new medicine as an example.[g]

3.4.1 Context

This is the 'introduction' section which explains the context and the submitter's **overall reasons** for asking for the medicine to be listed on the PBS. The submission first describes the target medical condition and the population for which the applicant (also called the 'sponsor') would like the medicine to be used. It also explains briefly how the medicine works in the body (i.e. the biology, chemistry and physics about the medicine). If relevant, it also needs to outline if submissions to the PBAC have been made for this medicine in the past. Of course, it needs to outline whether the TGA has approved it.

[f] Approvals are sought from the Department of Human Services (DHS) or Department of Veterans' Affairs (DVA) because these Departments are charged with administering the PBS and Medicare for general population (DHS), and war veterans, members of the Australian Defence Force, members of the Australian Federal Police, and their dependants (DVA)

[g] Sometimes a major submission could be for requesting listing of a new indication of an 'old medicine', or a new population for an existing listing.

As explained, comparison is at the heart of the way the PBAC considers a submission. So, a submission must identify a **comparator**, or multiple comparators, for which the new medicine can be weighed against in clinical and economic evaluation. To do this, the submission needs to first spell out what treatments are currently available in Australia to treat that same condition and in the target population. It then needs to explain how the new medicine, if listed on the PBS, would change the way that this condition or target population would be treated. For example, will this medicine be **added** to the overall treatment, will it **replace** the current treatment, or will it be an **alternative** option to existing treatment?

About a comparator(s)

- The PBAC defines a comparator as "the current alternative therapies in Australia, and the therapies most likely to be replaced in clinical practice".
- A comparator could be another medicine, surgery, ongoing care, or even 'dummy pill' if there was no medical management for that condition.
- TGA's ACPM usually looks at whether a new medicine is more effective and safe than placebo, whereas the PBAC often compares the differences against an existing treatment or therapy, unless there is no existing treatment.
- PBAC must assess a new medicine against a comparator.

One should note that the comparator doesn't have to be another medicine: it could be the standard medical management for the condition, which may involve surgery, ongoing care, or even 'doing nothing' if there was no medical management for that condition. Although the medicine is new, the comparator doesn't also need to be new. For example, a new blood-thinning medicine to prevent stroke could have warfarin and aspirin as the comparators even though warfarin and aspirin have been around for many years. Finally, the way the PBAC identifies a comparator is different to the ACPM. This is because the ACPM often looks at whether the new medicine is more effective and safe, often compared to a 'dummy pill' or placebo unless the trials present comparison to another treatment (they call these 'head-to-head' trials). In contrast, the PBAC often compares the differences in benefits and costs against an existing treatment or therapy, unless the treatment has no alternative treatment at the time when considering the submission.

3.4.2 Clinical evaluation

With the context specified, the PBAC now considers how good the medicine is in treating the condition in the target population compared to the nominated comparator i.e. comparative clinical effectiveness.[9]

Before being presented to the PBAC, manufacturers and their research collaborators have already invested millions of dollars to run many years of research to make sure a new medicine is safe and effective. The final phase of this series of clinical trials before being considered for regulatory and reimbursement approval (i.e. 'Phase III') usually, but not always, involves what is called a **randomised controlled trial,** or RCT. In this type of clinical study, researchers randomly assign patients into a treatment group to receive the medicine, and a control group to receive the comparator or a placebo. The most basic form of randomisation involves throwing a dice, tossing a coin, or drawing names out of a hat. Of course, clinical trials are more sophisticated nowadays and a computer usually does all the patient randomisation. By randomly allocating patients into groups, researchers are able to compare the outcomes of the trial participants, without worrying that the outcomes have been biased because people could non-randomly choose or be allocated a particular treatment they had preferred. As per scientific convention, the PBAC generally considers an RCT as the better study for evaluating the clinical effectiveness of medicines.

If patients are able to decide whether to take a certain medicine during a trial, the study is known as an **observational study.** This type of study can still be used in an application to the PBAC if carefully designed. However, it is not considered to be as good as a properly designed and run RCT because it is harder to say confidently that the observed benefits are due to the medicine itself rather than the characteristics of the patients who chose to take the medicine. But observational studies are sometimes the only possibly study option for reasons such as:

– it is not ethical to randomly assign treatment to study participants when it is known that the treatment may be more likely to be effective than the comparator; and
– when the disease is rare and the number of patients available to participate in the study is very limited.

Irrespective of the types of studies presented, the PBAC considers many other factors about the clinical studies before coming to a position about the comparative clinical effectiveness and safety. These include the number and design of the studies, the number of people in the studies (called the 'sample size'), and how researchers measure health outcomes, to name a few. After considering all types of information about clinical effectiveness and safety, the PBAC notes whether they are confident that the medicine, compared to the nominated comparator, is better (**superior**), at least equal (**non-inferior**) or worse (**inferior**) in changing the health of the patients. So, the decision making process requires the PBAC to apply a great deal of judgement. This makes the process they undertake part evidence, science and economics, and part application of judgment.

3.4.3 Economic evaluation

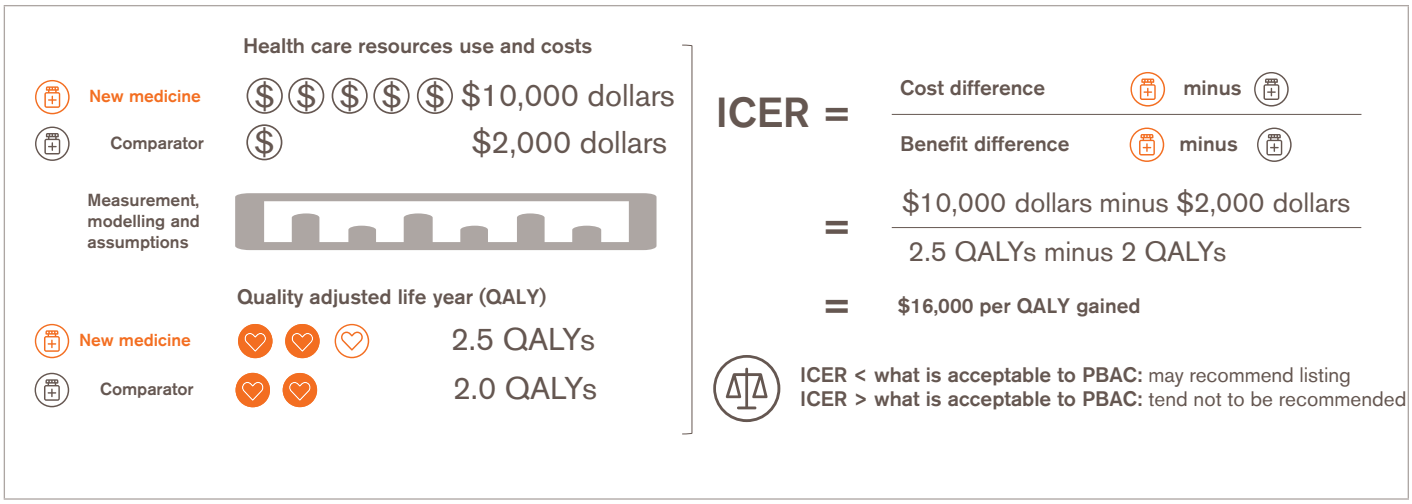
On the basis of the evidence on comparative clinical effectiveness and safety, the submission needs to present an evaluation of the economic benefits arising from the introduction of a new medicine to the PBS by determining its cost-effectiveness against the comparator. The economic evaluation is reviewed by the Economics Sub-Committee, who advises the PBAC on the quality, validity and relevance of submissions.[11]

The type of analysis will depend on whether the medicine is proposed as being superior, non-inferior or inferior to the comparator. When the proposed medicine is making a claim of non-inferiority, a **cost minimisation analysis** is required. In this case, all that is required is for the submission to first show a dose that is equally effective as the comparator selected. The new medicine is then priced at the same level as the comparator at the equivalent dose (i.e. "minimising" the cost to produce the same outcome). On the other hand, there are two scenarios when a **cost-effectiveness analysis** is required: (1) when a proposed medicine claims to be therapeutically better to the main comparator, but more costly to the health system; or (2) when the proposed medicine claims to be therapeutically worse than the main comparator, but less costly to the health system. Of course, it is a 'no brainer' that an **inferior** medicine that **costs more** than the comparator should not be listed and that a **superior** medicine that **costs less** should be listed.

Cost-effectiveness analysis involves **economic modelling.** Because the medicine is new and not yet available in Australia, a model is required to represent what would happen in real life to the patients and health system, for example, by drawing out the data from say three years from the RCT to predict the pattern in 10-20 years or even the lifetime of the patient. Like all models, these projections are based on a number of supportable assumptions, and hopefully these assumptions are reasonable and agreeable between the PBAC and the applicants.

To be precise, a model tries to work out the different benefits the proposed medicine would make to the health outcomes of the treated person and the costs associated with treating the condition, relative to the benefits and costs of the comparator, over a reasonable period. After a series of mathematical calculations, the model often summarises the health outcomes using a standard of measurement called a quality adjusted life year, or QALY. A QALY accounts for both the quantity and the quality of life, and can be used to compare medicines based on how long a person would live and the wellbeing of the person while alive. After comparing the costs, the model then calculates the extra cost per extra unit of QALY, known as the incremental cost-effectiveness ratio (ICER). An ICER essentially tries to suggest an answer to the question: are the extra (lower) health benefits from the new medicine the worth the extra (lower) cost?

Figure 3.2: Measuring the differences in costs and benefits of a new medicines and comparator to determine ICER



Once the PBAC has confidence in the evidence presented regarding clinical effectiveness and costs, and is satisfied that the calculations are all reasonable, the PBAC considers whether the estimated ICER is cost-effective. Some similar agencies in other countries have recognised 'threshold' ICER or standard on how much to pay for an extra QALY (e.g. $50,000 per QALY). It is common knowledge that the PBAC does not have a single 'threshold', and the PBAC considers a range of acceptable ICERs together with other factors, as discussed further below. Nevertheless, in simplistic terms, if the ICER falls below an acceptable 'threshold', the PBAC would be more likely to recommend listing, whereas those with an ICER above an acceptable 'threshold' tend not to be.

3.4.4 Use of medicine in practice

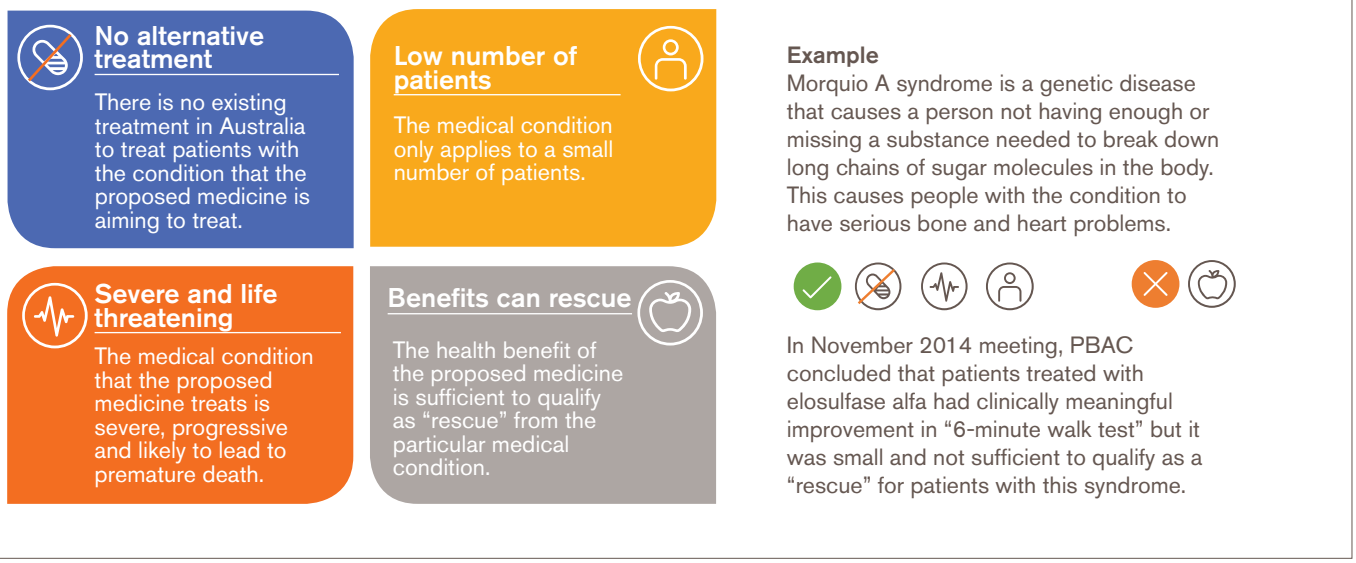
The PBAC is also interested in how much the medicine would cost the PBS budget and the wider health budget. So, the submission must provide modelling of the expected impact of the proposed medicine on both, considering the expected uptake of the medicine based on the number of people with the condition who would be treated with the new medicine if it were listed. Where the medicine is equal to its comparator, the submission needs to calculate the portion of the current market of the comparator that could now be taken over by the new medicine. That is, how much of the existing 'pie' would be switched from the existing medicine to the equivalent new medicine. The Drug Utilisation Sub-Committee assesses the forecasts provided by applicants for their validity and appropriateness.[11]

3.4.5 Other relevant information

The PBAC may also consider other information that would help them reach a more balanced decision.[9] This includes information on how the new medicine might support or hold back patient equity on access. If the new medicine is an antibiotic, the submission needs to show how the medicine might be used so that it wouldn't add more to the ongoing danger of causing antibiotic-resistant super bugs due to inappropriate use.

The PBAC may consider the use of the "rule of rescue" when they see a need to apply a sense of duty to help people with uncommon and very serious conditions where possible and without delay. In this case, the PBAC submission guidelines specify a set of four stringent conditions which, when met concurrently, can help the PBAC recommend the listing of a medicine (or not).[9]

Figure 3.3: Applying the rules of rescue, with an example where rules were not met



3.5 Post PBAC processes prior to listing

Once a medicine has received a positive recommendation, this does not mean it automatically goes onto the PBS.[9] First, there is a period of price negotiation between the Government and manufacturer and, if need be, updated modelling of the impact on the budget. This period may also involve negotiating what is called a 'risk share agreement' (RSA) between the Government and the manufacturer. This is usually in place when the medicine cost is high or when the PBAC has recommended that the medicine be subsided for specific patients only. Like warranties, properly designed RSAs can help to ensure that the medicine delivers value for money or to manage the risk of use outside of the expected patient population which may lead to unexpected or excessive expenditure on the medicine. Once the price has been established, the recommendation is sent to the Minister for Health who must approve it before it can become available to patients under the PBS.

Despite being an 'agreement', some industry stakeholders have considered the agreement as unbalanced if manufacturers bear more risk than the Government. These include having the manufacturers bear the full risk (i.e. paying the Government back) if the estimated cost to Government based on estimates of the number of persons with a specific disease, uptake rates, compliance and duration of treatment is exceeded. These estimates have been reviewed and sometimes decreased by the Drug Utilisation Sub Committee (DUSC) and PBAC, and may not reflect the manufacturers original estimates of use. In addition, the refund manufacturers provide to the Government as part of the RSA is going back to the government's overall coffer rather than to the PBS. There is an ongoing debate as to whether rebates should go back to the PBS budget to allow the listing of new medicines.

3.6 Post listing processes

Once listed, the usage and expenditure of medicines are monitored so that the modelled medicine usage can be updated to reflect what has actually been observed in real life once patients are actually accessing the PBS medicine. After 24 months, the Drug Utilisation Sub-Committee reports on the usage of listed medicines on an ongoing basis.[9] Depending on the findings, the PBAC may recommend that the minister revise the listing and access to a certain medicine or the type of approved prescriber, or it may recommend that a post-market review be undertaken. Post-market reviews are a systematic and formal approach to monitoring medicines listed on the PBS.

A post market review aims to contribute to:
- improved patient safety through better understanding of adverse events and medicine-related harms;
- ensuring the ongoing viability of the PBS through targeted medicines usage and avoiding; preventable wastage or inappropriate prescribing;

- a better understanding of medicines utilisation, to review intended clinical benefit and inform medicines evaluation processes;
- ongoing cost-effectiveness, including through better management of clinical and economic uncertainty;
- overall improvements to the quality use of medicines and education for patients and prescribers.

These reviews always involve consultations with relevant stakeholders and the expert reviewers would provide a range of recommendations to ensure the quality use of PBS listed medicines and the ongoing sustainability of the PBS. Previous reviews have made a range of recommendations. These include changes to improve quality use of medicines and reduction in price to realise better value for money. However, there has been inconsistent application of these recommendations. Some industry stakeholders have perceived the primary focus of post-market reviews is to reduce the prices of medicines.

3.7 When and how can consumers or consumer groups be involved?

Consumers are invited to make submissions for PBS applications before each PBAC meeting via the PBS.gov.au website. These are important so that the PBAC can consider any benefits and harms of any proposed medicines or vaccines, which may be distinct from those observed in the clinical evidence. Consumer submissions are considered at the same time as technical papers submitted by applicants.

Furthermore, there are several ways consumers can help provide access to medicines. These include:
- asking your healthcare provider about clinical trial options that may be appropriate for you;
- providing your view to the PBAC on new medicine listings at PBS.gov.au;
- monitoring PBS reforms in Parliament and telling policymakers what you think at APH.gov.au; and
- learning more about the system and staying connected with consumer, healthcare and medicines peak bodies.

3.8 Listing vaccines on the PBS

The PBAC also considers submissions from manufacturers to list vaccines on the **National Immunisation Program** (NIP)[11]. It follows more or less a similar process but there is much more planning involved both before submission to the PBAC and after PBAC recommendations for the vaccine to be added to the NIP. The NIP is an established collaborative program involving the Australian Government and the state and territory governments, which aims to increase national immunisation coverage rates by funding essential vaccines for eligible infants, children, adolescents and adults.

Prior to consideration by the PBAC, new vaccines or new vaccine programs are first considered by the Australian Technical Advisory Group on Immunisation (ATAGI). ATAGI first assesses what is called ‘horizon scanning’ to see if there are potentially important developments relating to infectious disease and vaccines. ATAGI works with medicine companies and the Department of Health. It then provides advice to the PBAC on vaccines, in addition to providing advice to the Department of Health and Minister on matters relating to immunisation.

The manufacturer of the vaccine then provides a submission for the PBAC’s consideration, as described in Sections 3.2 and 3.4.

Once a vaccine has received a positive recommendation from the PBAC, the Office of Health Protection seeks approval from the Minister to fund the vaccine under the NIP. A long period of post-PBAC program planning ensues, and it involves:

- negotiations between States and Territories;
- modification to the Australian Childhood Immunisation Register, if relevant;
- confirming a communication strategy;
- amendment to legislation;
- formulating Vaccine Safety Monitoring Plan;
- conducting tenders to procure supply of the new vaccine for the NIP; and
- planning for disease surveillance and adverse event monitoring.

Because of this additional planning, the listing of vaccines on the PBS or NIP usually takes much longer than the listing of medicines.

It is important to note that vaccines work in a different way to medicines. When a person is vaccinated, their body produces an immune response in the same way their body would after exposure to a disease or infection. However the person is not likely to suffer symptoms of the disease or infection. If a person comes in contact with that disease or infection in the future, their immune system will respond fast enough to prevent the the development of the disease or infection in that person. When vaccination rates across the community are high, this can also bring benefits for the entire population through a concept known as "herd immunity". More information is available at http://www.immunise.health.gov.au

4 Pricing of PBS medicines

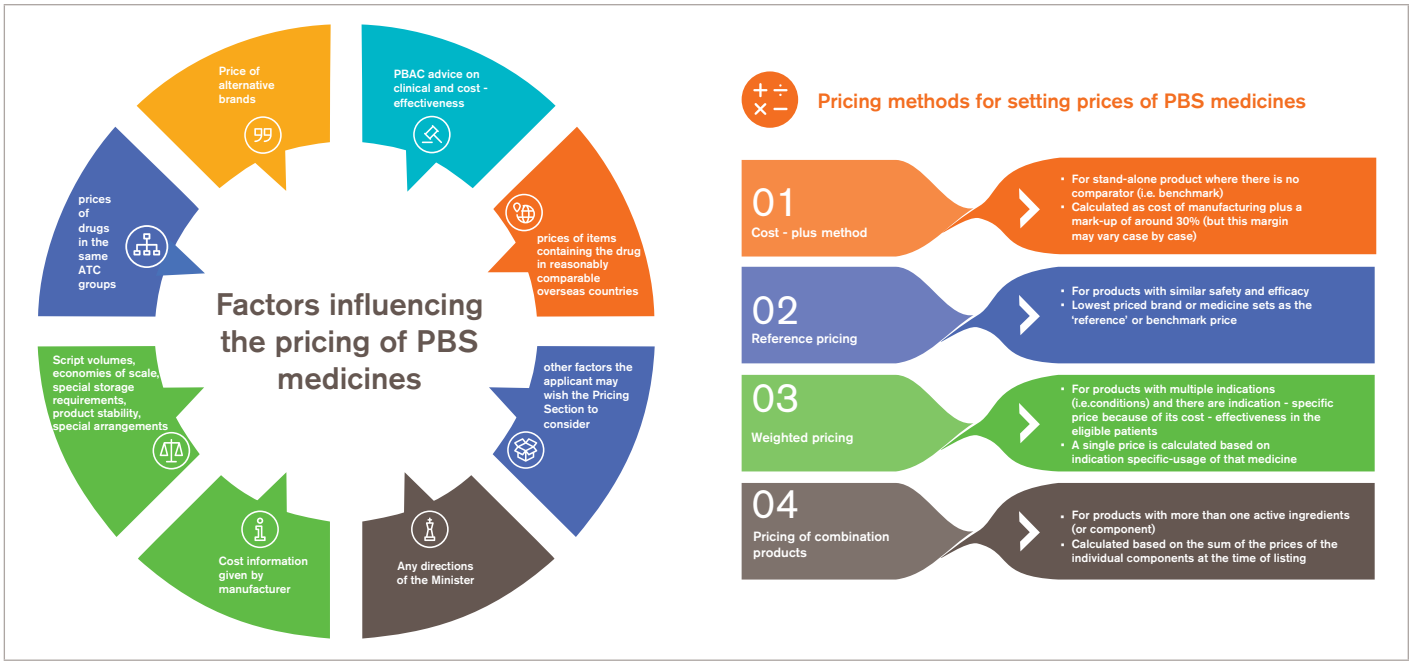
4.1 Overall principle of pricing PBS medicine

Upon receiving a positive recommendation for a new listing on the PBS from the PBAC, the Pricing Section of the Department of Health enters into negotiations with the medicine manufacturer regarding pricing and, if required, any risk share agreement. Sometimes, the PBAC recommends pricing negotiation to lower the price to meet the cost-effectiveness criterion. Using a medicine for hepatitis C (sofosbuvir) as an example, the PBAC's positive recommendation was subject to further price negotiation and a risk share agreement (see clauses 6.27 and 6.39).[12]

The Pricing Section considers a number of factors when considering the price of medicines and reviewing the prices of existing PBS medicines. These include PBAC advice on clinical and cost effectiveness, prices of alternative brands and medicines in the same 'therapeutic group' (as classified in the so-called ATC classification system), and so on (Figure 4.1). The Pricing Section also uses different methods to set prices for PBS medicines according to the product type. These include cost-plus method, reference pricing, weighted pricing and a specific form of weighted pricing for combination products.

The following section explains these methods and other pricing conditions that apply once a product is on the PBS.

Figure 4.1: Factors and influencing the pricing of PBS medicines, and the different pricing methods



Source: PBS factsheet – Setting an approved ex-manufacturer price for new or extended listings

4.2 Setting the price for PBS medicines

As explained in Section 3.4.1, when considering a listing, the PBAC weighs the relative clinical and economic merits of a new medicine against a nominated "comparator" or multiple comparators. So, if the submission receives a positive recommendation, the price will depend on how well the medicine performs in comparison to the comparator as advised by the PBAC.

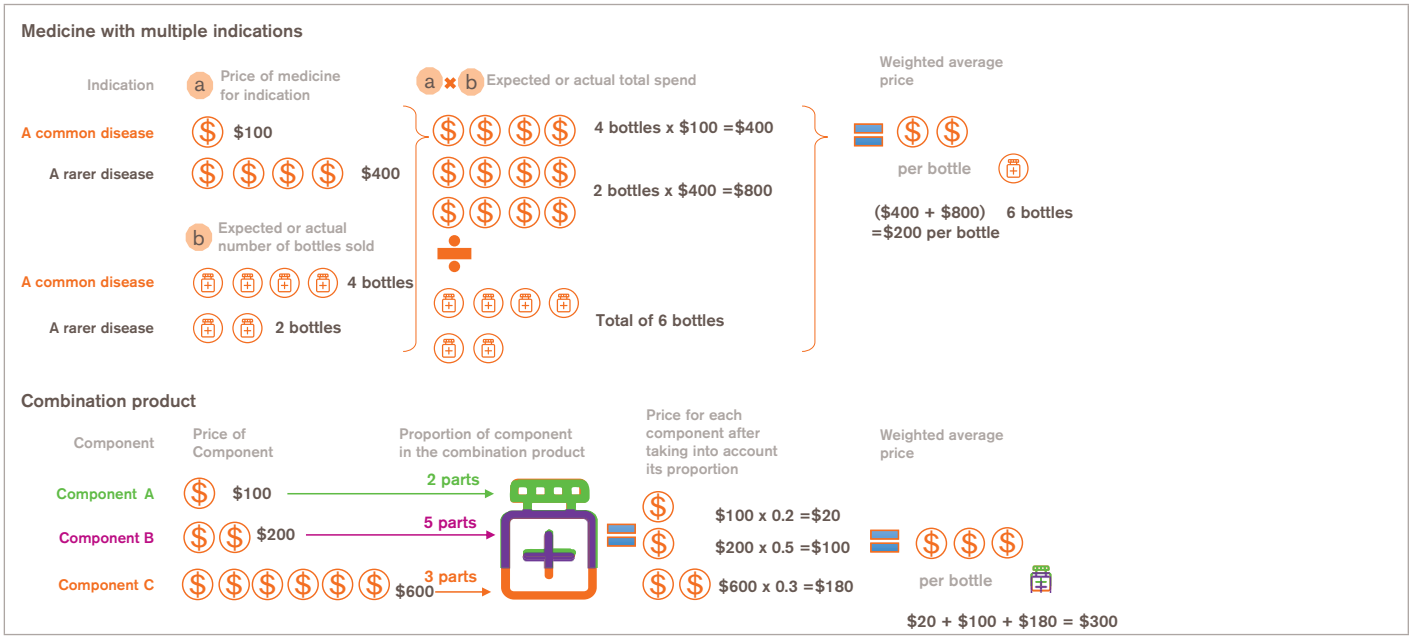
If the medicine is **equivalent or non-inferior** in health outcomes, the Pricing Section applies a **cost-minimisation** approach. This involves setting the price of the new medicine equal to the least expensive option of the medicine in the comparison group that offers the most similar level of effectiveness to the new medicine. This is sometimes referred to as "Reference Pricing". It reflects how much consumers usually expect to pay for a product in relation to other competitors and the previously advertised price: if a new product is as good as the alternative, why pay more than the least expensive option? However, it should be noted that some individual consumers may have better or worse outcomes from medicines that are considered clinically equivalent on average across the whole target population. For example, they may have side effects from the old medicine but not from the new one. So, patient choice is also important at a personalised level.

If the medicine is **superior or inferior** (i.e. better or worse) to existing therapies in the comparator group, then prices will be set relative to the effectiveness of the existing therapies. The PBAC publishes **"Therapeutic Relativity Sheets"** to inform pricing according to cost-effectiveness requirements. So, the price of the new medicine may be higher or lower than the prices of comparable existing therapies depending on the relative differences in benefits.

The way of pricing is more complicated for medicines appearing multiple times on the PBS for a set of approved conditions and patients and for combination products. Under the current law (*National Health Act 1953*), there can only be one published price for a pharmaceutical item (i.e. medicine with a specific pack size and dosage). So, when a medicine has different prices for different conditions due to different size of benefits for different conditions and cost-effectiveness, the Pricing Section applies what is called "**weighted pricing**". This means that they apply different weights to each indication-specific price according to observed or expected volume of use for that medicine and indication, and then add these prices together in order to arrive at a single weighted price. For example, as shown in Figure 4.2, a medicine costs $100 for a common disease but $400 for a rare disease according to its cost-effectiveness. It is expected that the medicine would be sold twice as much for the common disease (say four bottles) compared to the rarer disease (two bottles). Based on this, the weighted average price per bottle is $200 ($1,200 divided by six bottles).

Combination products with multiple components use a similar mathematical principle to arrive at a single price. As illustrated in Figure 4.2, if a combination product contains two parts of component A at $100 for each part, five parts of component B at $200 for each part, and three parts of component C at $600 for each part, the weighted price for the combination product is $300 ($3,000 divided by 10 parts).

Figure 4.2: Methods used for setting the prices of PBS medicines with multiple indications and combination products



4.3 Pricing of medicines once on the PBS

Normally, just like other technology related products (e.g. computers and mobile phones), one would expect the prices of the same medicine to fall over time as these medicines are replaced by newer and better medicines. On the other hand, the costs of manufacturing and distributing the same medicine may increase over time because, for example, the salary of the workers have increased or the costs of replacing manufacturing equipment have increased. As discussed, the Government sets prices of PBS medicines at the time of listing and the prices would be the same (without adjustment for potential increase in manufacturing and distribution costs), until policies that sought to reduce the prices kicked in. As mentioned in Section 2.3, the Government has worked together with industry stakeholders to reorganise the PBS quite a number of times (and they call it "reform") to make sure the system remains financially healthy and the pricing mechanism resembles how prices change in the market.

Over the years, the reforms have resulted in a set of pricing rules for medicines listed on the PBS. These include **statutory price reductions** at various time points from the time of listing, and price reductions due to **price disclosure**. It also includes **price linkages** between different medicines belonging to the same therapeutic group, and between combination products and their component medicines.

Before explaining these pricing rules, it is important to mention again the three lists of medicines within the PBS, which are called "formularies":

- **Formulary 1** (F1) consists of single-brand medicines, usually on-patent;
- **Formulary 2** (F2) consists of multi-brand medicines, which are often "generics" and biosimilar medicines; and,
- **Combination Drugs List** (CDL) consists of combination products with a single brand. This category is similar to F1 except that some of the component medicines may be in F2.

Under the current policies agreed between the Government and the medicine industry, the prices of medicines on F1 are generally stable and 'protected' from price reduction **except** the 5% price cut as required by law (i.e. statutory price reduction) when they reach the 5th "birthday" on the PBS. This allows manufacturers to operate with a degree of market control and be able to maintain the listed price while the medicine is under patent. This is to ensure that manufacturers are given a time period to generate return for their investment on their intellectual property.

> *So, intellectual property reflects an economic trade-off, a balancing act. If it's too generous to the creators, then good ideas will take too long to copy, adapt and spread. If it's too stingy, then maybe we won't see the good ideas at all. This trade-off has always been coloured by politics.*
>
> *Tim Harford*

However, the Government may still apply price reductions to F1 medicines based on therapeutic relativity – a concept explained in Section 4.2. In this case, suppose Medicine A is already listed on F1 at $1,000 per pack. The manufacturer of Medicine B – a new medicine considered as therapeutically equivalent to Medicine A – now seeks PBS listing at a price of $900 per pack. If approved by the PBAC, the Department will ask the manufacturer of Medicine A to reduce its price to match Medicine B at $900. So, although prices are more stable on F1, it is not fully shielded from price reduction.

If another brand of the same drug enters the market, the medicine is moved to F2, as with all medicines for which two or more brands exist. When this happens, there is a one-off price reduction of 16%. Once in F2 where there are multiple-brands of the same medicine, market competition usually drives prices down. To reap the benefits of this competition, a pricing rule called "price disclosure" kicks in. In this case, manufacturers are required to disclose to the Department of Health their ex-factory prices. If the average ex-factory price is more than 10% below the agreed PBS price, then the agreed price will be reduced to the same level. This avoids a situation where the Government would otherwise end up

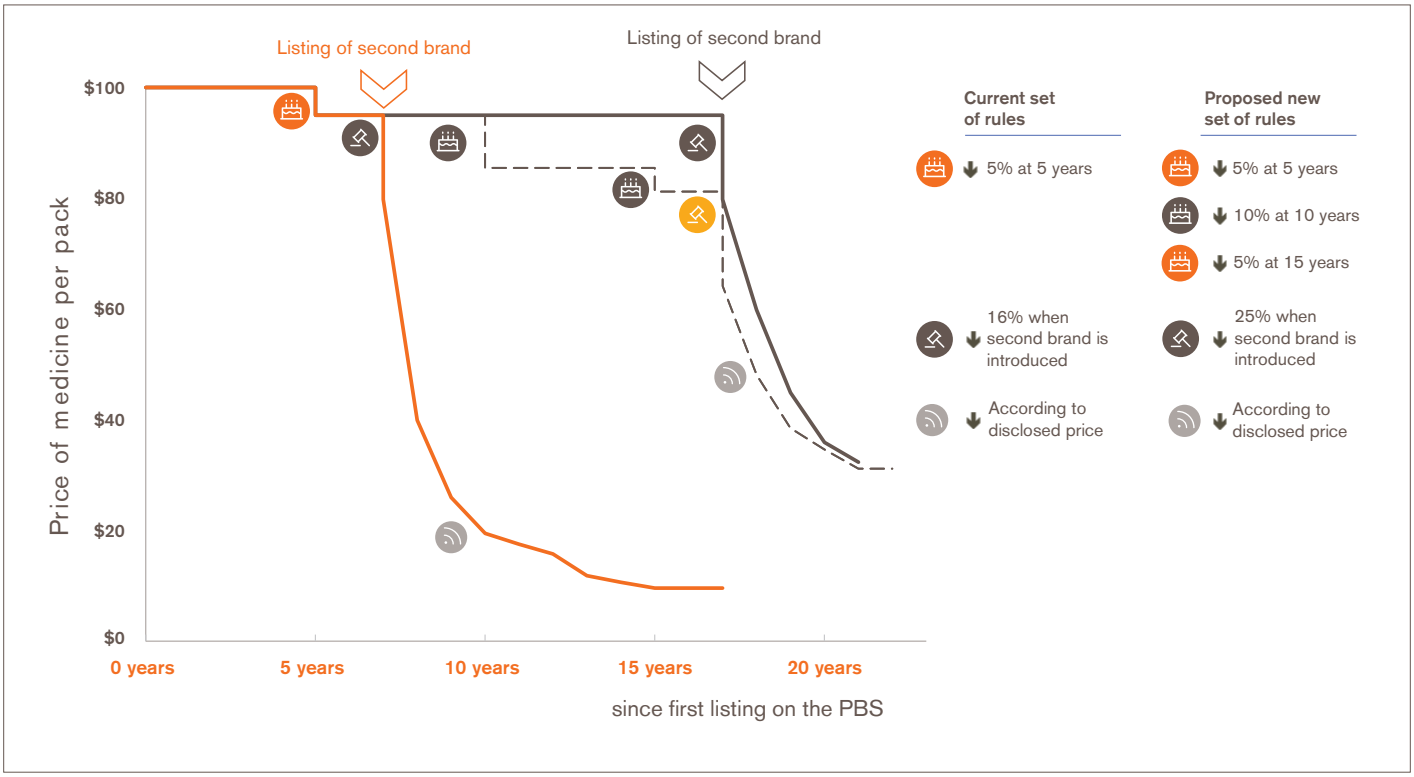
subsidising medicines for more than the price at which they are actually being sold. Figure 4.3 shows the effect of the current pricing rules applied to an imaginary medicine priced at $100 when it is first listed on the PBS, which does not have a second brand of the same medicine listed until 17 years since its first listing. In reality, some medicines would have come off patent earlier, as shown in the orange line in Figure 4.3.

Price linkages are applicable to specific sets of medicines listed in F2 that are of similar safety, efficacy and provide similar health outcomes (i.e. medicines that are interchangeable within a therapeutic group). For example, as of 2017, the PBS specifies two therapeutic groups: a group of medicines for heart and blood pressure called "angiotensin II antagonists"; and another group of medicines for acid reflux called "H2-receptor antagonists". Price linkages within therapeutic groups mean that if the price of one medicine is reduced, then the price of other similar medicines will also be reduced. The separation into two formularies, though, means that newer medicines in F1 don't have their prices reduced when the price of multi-brand medicines in F2 fall.

However, the prices of single brand combination products listed in CDL are linked to their component medicines in F1 and F2. Using the combination product example in Figure 4.3, if component B is in F2 and experiences price reductions due to market competition, the price of the combination product in CDL will be adjusted even if there is only one brand for that combination product.

Figure 4.3 also shows the effect of additional pricing rules proposed as part of the Government's 2017-18 budget announcement. As mentioned in Chapter 2, these proposed changes would require approval from the Australian Parliament before they would come into effect.

Figure 4.3: Current and proposed pricing rules for medicines listed on the PBS



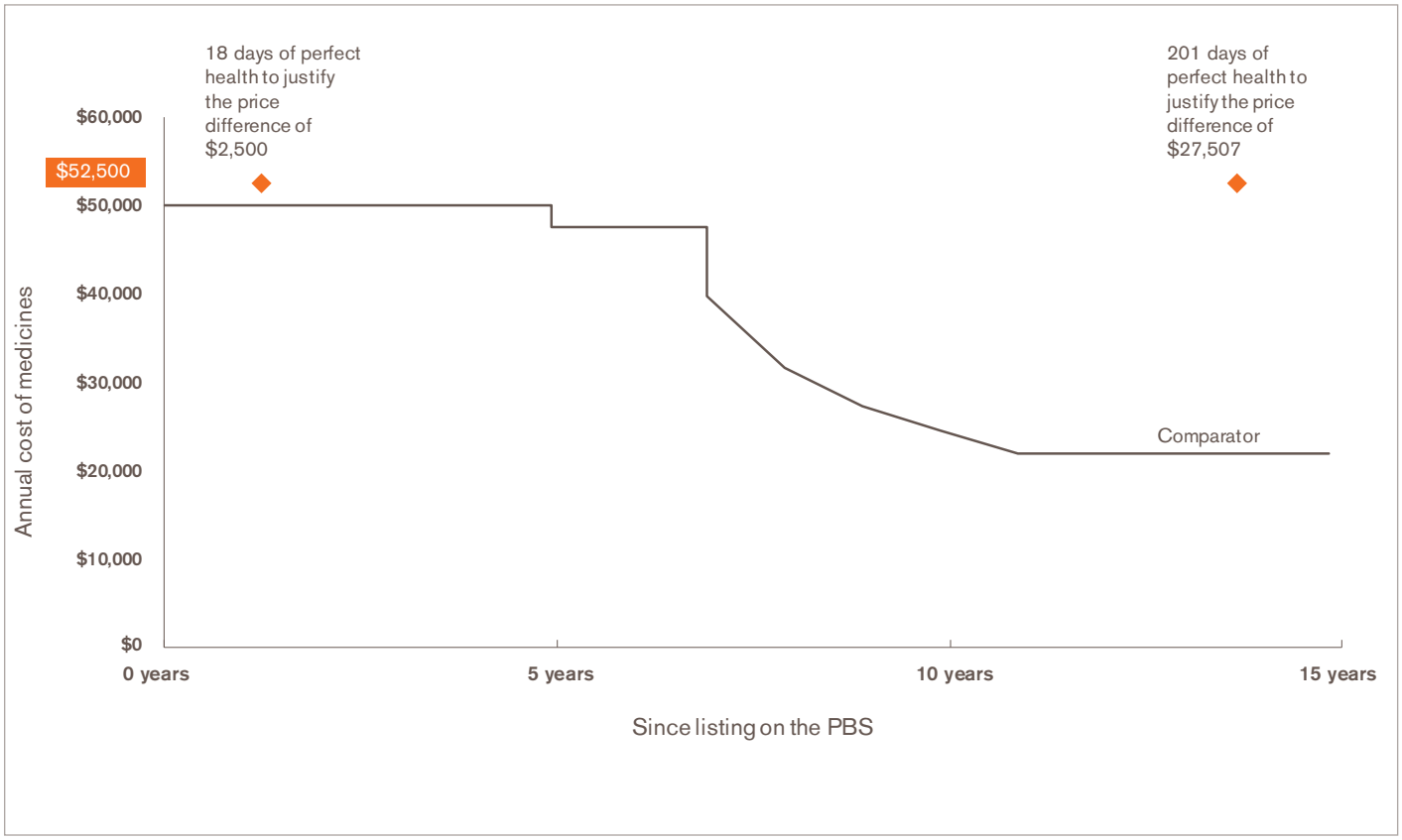
4.4 Two potential tension points with the current pricing rules: views from the industry

While recognising the importance of having a sustainable PBS, the medicine industry has noted a few potential tensions with the way prices are set when listed and managed after listing.

One of these tensions arises when the identified comparator is a medicine that has been on the PBS for many years. All the rules to reduce prices and competition applied over the years mean that the price of this comparator medicine could be very low. This has been referred to as "**comparator price erosion**". Being benchmarked to this comparator, the new medicine seeking listing on the PBS would often need to show very large gains in health outcomes in order to satisfy the PBAC's threshold for cost-effectiveness. Naturally, the medicine industry would love to discover and make new medicines that offer huge improvements on the health outcomes to patients. But medicines like this are hard to come by and most improvements build up incrementally in small steps over time, rather than in big leaps.

Figure 4.4 presents an example to show the potential issue due to comparator price erosion. Let's say a submission is seeking the listing of a new medicine priced at $52,500 per patient per year. The amount of health benefits the new medicine would need to demonstrate in the submission depends on the timing of the submission. If the submission is lodged two years after the comparator has been listed on the PBS, the difference in price is $2,500 (i.e. $52,500 minus $50,000). With this difference, the new medicine only needs to show around 18 extra days of perfect health to meet an assumed PBAC threshold of $50,000 per QALY. However, if the comparator has been listed on the PBS for 10 years and its price has been halved to due to the pricing rules, then the new medicine would need to show around 200 days of **perfect** health to justify the price difference of round $27,500 to meet the assumed PBAC assumed threshold. Patients with very severe illnesses may not have much capacity to improve, but a small improvement, even 1-2 months of extra life for example, means a lot to them if they only expect to live for another 6 months. For this reason, if the comparator has been on the PBS for a long time, the bar for the expected improvement in health for the new and better medicine to demonstrate would be too high. This may have an impact on patient access to new medicine.

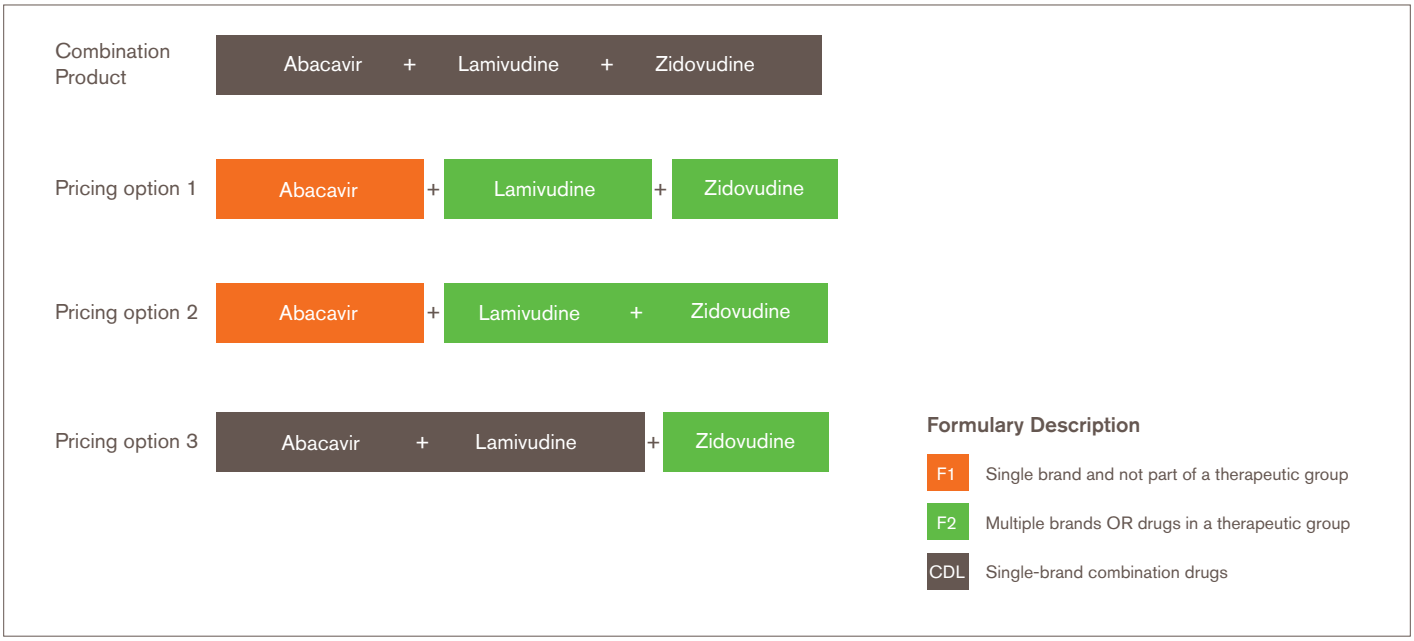
Figure 4.4: Different health gains when benchmarked against the same comparator at different time points



A second potential tension relates to the pricing of combination products. Figure 4.5 shows the three options to calculate the price of a combination product with three component medicines: abacavir, lamivudine and zidovudine for HIV. To complicate things further, the component medicines also come in other combination products with two component medicines: lamivudine with zidovudine and abacavir with lamivudine. All these components and combination products are in different formularies, as shown. Under the current pricing rules, the prices of all these products are linked and the price of the combination product would be determined based on the lowest price from the three pricing options.

For the manufacturer to bring this combination product to PBS, it must fulfil all the regulatory requirements by TGA and a major submission to the PBAC. Despite this investment in bringing a medicine that may offer significant convenience to patients or better health outcomes, its price is linked to so many products which makes it difficult for the manufacturer to predict how the price of this combination product will be reduced. Much like any other market, unpredictability and falling prices can make supply and pricing difficult to manage for manufacturers. This may create pressure on sustainable long-term access to this combination medicine.

Figure 4.5: Pricing of a combination product with various pricing options



5 Stakeholder views

Eighteen stakeholders representing various patient organisations and peak bodies (Appendix A) were consulted to understand their views on the PBS. During the consultations, stakeholders were asked to consider various points of tension in the system that arise because of the system's complexity and the associated competing needs. These tensions include the relative importance of decision making criteria (see Figure 3.1) and accommodating individual consumer preferences in a national scheme and so on. Stakeholder views, centred on these tensions, are summarised below.

5.1 Decision making

Stakeholders were asked to give their views on the decision making criteria used to determine whether or not a medicine should be listed on the PBS.

Overall, stakeholders acknowledged the privileged position Australians have in accessing affordable medicines relative to other developed nations. Many stakeholders expressed their gratitude that the Australian Government negotiates to achieve a good deal for the health system and patients.

Stakeholders deemed the PBAC's focus on budgetary control reasonable to limit costs to the taxpayer. Many stakeholders acknowledged that the Government would not be able to afford every medicine. For this reason, they tend not to expect the Government to accept every price requested in the submission by the manufacturers. The decision making criteria was considered mostly reasonable, especially the evidence based approach in decision making.

Despite their appreciation, many stakeholders have highlighted the main points of tension to be discussed below. They have also pointed out areas the PBAC may consider to improve the decision making process.

5.1.1 Budgetary control vs providing access to new medicines

Stakeholders aligned to the view that budgetary considerations should be made while considering the whole health budget, and not just the PBS component. They had the perception that the current PBS system needs to find savings within the system or to delist older medicines to make way for the listing of newer medicines. Some of them perceived the PBS system as "one in, one out" and they considered this inappropriate.

Some stakeholders believed there was more room for flexibility in the PBS budget, particularly for truly innovative, or "game changing", medicines. If there was a choice between (a) funding an expensive but life-saving medicine, versus (b) delaying access in order to drive a better deal with manufacturers or searching for savings elsewhere, stakeholders erred on the side of providing immediate access. This was premised on stakeholders' beliefs that access to medicines is often time-critical: an extra month without treatment can be the difference between life and death for a patient. An unwanted side effect of such delays pointed out by stakeholders was that it could dis-incentivise manufacturers to attempt listing in Australia, or even hinder innovation altogether.

In the case of rare diseases affecting a small number of patients, some stakeholders thought there was a need for an alternative mechanism for listing. The current process was seen as being geared towards more prevalent conditions, due to the need for clinical evidence. Stakeholders considered that the technical requirements can often be more difficult to produce when the target population is limited. In these special cases, stakeholders thought that new medicine should be made available to the eligible population based on lower requirements. Some stakeholders even perceived that the PBS may not be the most appropriate mechanism for providing access to medicines for rarer medical conditions.

5.1.2 Balancing personal preference in a national scheme

While stakeholders' support for budgetary control is seemingly at odds with their support for providing quick access, stakeholders pointed out that a person's perspective on the matter could change. This was premised on the notion that if they switched from being a taxpayer to someone actually requiring medicines, their perspective may change. One stakeholder stated that "as a taxpayer I would like to see the evidence base … but as a patient I would like have access to medicines at a subsidised rate." They judged that as it stands, the PBS listing process puts too much weight on the taxpayer's perspective, and may not have enough consideration on the side of the patient.

In a related discussion, some stakeholders felt there is currently too much emphasis on improving quality of life (e.g. when the PBAC mentioned that they have seen toxicity from some new medicines) over length of life. To them, this may not align with every patient's preferences. One example given was that for a given cancer diagnosis, one patient may prefer the maximum extension of life to spend time with their children even if it means that they would suffer from severe side effects. But other patients may simply want a better quality of life, even if for a shorter length. The current system does not leave this choice to the patients and their treating doctors. Some stakeholders shared the view that patient choice could therefore be expanded.

5.1.3 Technical analysis versus human dimension of providing access to medicines

Many stakeholders stated that the PBAC currently puts more weight on the more technical aspects of the decision criteria, such as the clinical evidence and economic modelling, while playing down the importance of the human aspects, such as equity and quality of life. While they were supportive of an evidence-based approach to listing in order to avoid the taxpayer spending money on treatments that are not cost-effective, they emphasised the need to reflect the human elements of access to medicines in the listing process. So, patient awareness of the QALY consideration already involved in PBS access could potentially be increased to improve understanding of the human elements in the decision.

> *Many times the patients know that PBAC is working with a limited "pot" of funds; they are unable to understand the reason why the drug they are interested in does not get listed and a number of others do.*
>
> *A stakeholder*

Stakeholders noted there was an opportunity for two-way communication flows between patient groups and peak bodies, and with the PBAC. In many cases, patient groups would only find out about a relevant medicine starting the PBS listing process from the manufacturer. They thought that the PBAC could do a better job of informing them about upcoming requests for listing a new medicine and soliciting submissions from patient groups themselves. They also wanted more information and notification from the PBAC regarding when patient input would be accepted during the listing process, and for this to be made more visible to both patients and patient groups. There was a suggestion that a fact sheet or flow chart could be developed with rough timelines detailing the step-by-step process that clearly marks the points in the process whereby consumers could contribute to the decision making process.

Stakeholders pointed out that patients themselves were quite often even less informed than patient peak bodies, and that this could lead to frustration or poor decision making. For example, due to the ease of accessing information via the internet, patients were increasingly informed about new medicines available elsewhere in the world, some of which had even been already approved for use in Australia by the TGA. What they lacked was any reason or explanation for a medicine failing to be listed on the PBS, which was blamed on the opaqueness of the public summary documents released by the PBAC. These were complicated for patients to understand due to the use of technical terms. Furthermore, these documents lack enough detail for patients to understand why exactly a medicine was rejected

for listing. To improve transparency, stakeholders suggested that the PBAC should release more accessible summaries of the reasons for rejection, for example including the reasons *why* there was uncertainty in the evidence presented or *why* it was considered cost-ineffective, rather than just stating that there was insufficient evidence. In the event that a medicine was rejected, consumers would like to know what would be the next steps and when they could potentially expect access to that medicine.

5.2 PBS pricing measures

While stakeholders had firm opinions on the various decision making criteria used by the PBAC to determine which medicines are listed on the PBS, the various pricing mechanisms imposed once a medicine is listed were less well understood.

5.2.1 Savings from statutory price reduction and price disclosure

In general, stakeholders had a limited understanding about the various price cuts a medicine undergoes subsequent to listing, including statutory price reductions and reductions through price disclosure.

When presented with a clear explanation of these various pricing rules, stakeholders supported the reductions from the perspective of minimising budgetary impacts to the PBS to allow newer and more expensive medicines to be made available. The price disclosure policy was deemed a reasonable and fair action for the Government to have taken, to avoid paying more in subsidies than pharmacists were being charged.

5.2.2 Comparing prices to older medicines

Stakeholders were new to the concept of price linkages between old and new medicines. In most cases, stakeholders were initially unclear that in many cases new medicines might not be better than what is already available on the PBS (for instance they may have different active ingredients, but with the same effects), and that this is the rationale used by the PBAC to anchor prices to existing medicines. Once stakeholders understood this rationale, they were generally supportive of the cost-minimisation approach, agreeing that for budgetary reasons it is sensible for the Government to avoid spending more money for the same outcomes.

During the consultations, stakeholders were presented with an example which showed that Medicine A was initially listed at $100, but only costs $10 ten years later due to price reduction measures, including those resulting from reductions in linked comparator products. If a new Medicine B were to apply for listing, the consensus view was that a higher price tag than $10 would be acceptable if there were demonstrable benefits for Medicine B. Some advocated the price for Medicine B should be in line with the original $100 price for Medicine A, albeit with some indexation taking into account changes in inflation and manufacturing costs, but most agreed that prices should be set relatively higher or lower in accordance with the relative effectiveness of the medicine, in order to reward incremental innovation. However, stakeholders were wary of designing the system in such a way that genuine innovation would be compromised, such as pricing at a level insufficient to incentivise producers.

In general though, stakeholders supported the idea of the need to incentivise incremental innovation. i.e that that manufacturers should have incentives for incremental benefits with incremental price increases, to a point. That is, medicines that have incremental benefits should be rewarded for not just the efforts of the innovation but also for the benefits that innovation brings to patients. Stakeholders were of the view that even if a lot of research and development went into the development of a new medicine, if it brings no perceivable benefit over an existing medicine, it should not receive a higher price. It was pointed out that this would lead to more focused innovation: funding pathways for innovative drugs should be based on the improved health outcomes, to encourage the development of medicines with greater incremental benefits.

5.2.3 Price linkages to combination products

Many stakeholders were new to the complexities of price linkages for combination products. Most agreed that benefits provided by combination products should be paid in the form of higher prices, but not significantly so. To an extent, combination products were viewed as more of a convenience, and stakeholders acknowledged that consumers ought to pay a bit more for the convenience, though it was debated whether such conveniences ought to be funded by the Government rather than the consumer.

Stakeholders generally agreed that where combination products provided a real and tangible benefit over taking each component separately, they ought to be paid in some form, and that it was appropriate for the PBS to fund this. While the PBAC already pays for combination products that are likely to improve script compliance, some stakeholders argued that any benefits that provide a tangible benefit to quality of life should also be paid in the form of a PBS subsidy.

Some stakeholders made the point that pharmaceutical manufacturers, just like other companies, should assume some of the risk of a product not commanding a high degree of market share (i.e. a business risk). Incremental improvements would be rewarded through higher market share, and stakeholders pointed out that this would, to an extent, offset manufacturers' concerns about not receiving a high enough price if they were developing improvements that consumers would want to pay for.

5.3 Summary

Stakeholders were generally supportive of the PBS decision making process and the system as a whole. However, they outlined a range of tensions they perceived with the current with.

- The decision criteria may currently be weighted too heavily on the side of population health outcomes, with not enough flexibility for patient choice and to accommodate individual preferences.
- Decisions appear to be budget-focused, which was deemed a prudent quality from the taxpayer's perspective, but at the same time meant patients might be cut-off from new treatments in a timely manner.
- It was perceived that the PBAC pays more attention to clinical evidence and economic modelling; and less attention to patient choices or needs.
- Mandatory price reductions seem appropriate.
- Cost-minimisation is appropriate for medicines with similar benefits.
- Medicines that offer demonstrable incremental improvements should receive incremental increases in price, but the PBAC should only accept the price when it is commensurate with the benefits.
- Combination products that offer a quality of life improvement, or improved patient outcome should be rewarded for doing so, but again, the PBAC should only accept the price when it is commensurate with the benefits.

By highlighting these tensions, stakeholders wish to inform considerations for future policy development so that Australians can continue to have timely and affordable access to medicines through a sustainable PBS.

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Appendix A

List of organisations that participated in consultation

Allergy and Anaphylaxis Australia
Australian Federation of AIDS Organisations
Breast Cancer Network Australia
Haemophilia Foundation
Hearts4Heart
Living Positive Victoria
Lung Foundation Australia
Melanoma Patients Australia
Myeloma Foundation of Australia
National Association of People with HIV Australia
National Asthma Council
Parkinson's Australia
Positive Life NSW
Victorian AIDS Council

GSK and ViiV Healthcare would like to acknowledge and thank the above organisations for their involvement in this consultation.