02. The Pharmaceutical Benefits Scheme

Medicines Australia

Better health through research and innovation

The Pharmaceutical Benefits Scheme

> Since 1948 the Pharmaceutical Benefits Scheme (PBS) has provided Australian patients with access to affordable and high-quality, safe and effective medicines when they need them.

Underpinned by the National Medicines Policy (NMP) and a strong working relationship between Government and the medicines industry, the PBS has evolved from basic access to antibiotics and painkillers, to a sophisticated system that that has had to contend with changing burdens of disease and evolving innovations in treatments. Examples of the most recent adaptations include:

1. **Agreement to develop and adopt streamlined pathways to improve and speed up PBS listing processes, delivering timely access to medicines, and**
2. **Commitment to review the National Medicines Policy to ensure it remains fit for purpose to accommodate emerging health technology breakthroughs.**

The PBS has a strong reputation within and outside Australia for being one of the best systems available to both patients and taxpayers. It works as part of the national, publicly funded, healthcare system whereby the government subsidises the cost of approved prescription medicines to make them available and affordable for the Australians that need them. Patients are required to contribute a so-called co-payment of $40.30, or $6.50 for concession card holders. The government pays for the cost of the medicines, as well as for the distribution and associated dispensing activities.

In 2017-18, over 204 million prescriptions were subsidised through the PBS, highlighting the key role this system plays in healthcare for Australians[1]. A large proportion of Australian patients access the PBS through concessional arrangements.

Under the National Health Act 1953, for a medicine to be added to the PBS, it must be assessed and recommended by the independent Pharmaceutical Benefits Advisory Committee (PBAC). In fact, the Minister for Health (or their Delegate) cannot list a medicine on the PBS without a positive recommendation from the PBAC. A submission to consider a new medicine on the PBS can be made by industry sponsors of medicines and medicinal products, medical bodies, health professionals, and private individuals and their representatives. However, they are most commonly made by the pharmaceutical sponsor or manufacturer, as they have the scope of evidence required to support such an application.

What does it mean for the Australian Government to be committed to universal access to healthcare?

The purpose of a national, publicly funded, universal healthcare system is to ensure that there is equitable access to health and medical goods and services, wherever you are located and regardless of your socio-economic status.

It means providing a system that reduces financial and geographical barriers for patients. It also means ensuring timely availability to the most appropriate innovations in medicine, pharmaceuticals and vaccines.

In a consumer survey commissioned by Medicines Australia, 85% of respondents agreed that everyone has a right to affordable medicines and half agreed that they would like to see more subsidised medicines in the future.[2]

The PBS Today

> The PBS operates within the policy framework of the National Medicines Policy (NMP), which incorporates four goals:

1. **Timely access to the medicines that Australians need**
2. **Medicines meeting appropriate standards of quality, safety and efficacy**
3. **Quality use of medicines**
4. **Maintain a responsible and viable medicines industry**

In collaboration with the innovative medicines sector, successive governments have pursued reforms to the PBS to ensure the healthcare system is adaptable to patients' changing healthcare needs. The NMP has been in place since 1999 and the innovative medicines sector agrees that a review of the NMP would be appropriate to ensure it remains fit for purpose to enable continuing patient access to new and innovative medical breakthroughs as they become available.

As a mechanism for subsidising patient access, it is also imperative that medicines access remains financially sustainable.

Reforms often focus on delivering savings and proving the value for money to Government and to the Australians who use the system, with some effort allocated to improving the processes for the listing of medicines. It is vital that any reforms to the PBS and how medicines, vaccines and emerging therapies are accessed, are drawn from strong consultation with industry due to the lengthy time periods involved in bringing medicines to market.

The PBS relies on a complex and highly interdependent supply chain; from the medicines manufacturer who invents, discovers, develops, manufactures and supplies a medicine, to the medical and health professionals who diagnose, select and prescribe a medicine, and the network of wholesale distributors and community, hospital and compounding pharmacy outlets who dispense the medicine to the patient. This complex supply chain ensures that Australian patients can receive the medicines they need, when they need them.

#1 The Medicine Approval Process

> The process for getting medicines from the laboratory to the patient is lengthy and complex.

During the discovery, scientific, and clinical development of a new medicine, the sponsor collects and analyses all the data, and uses this to build a package of evidence that demonstrates the quality, safety and efficacy of the new treatment.

To achieve medicines approval in Australia, the sponsor must first apply to the Therapeutic Goods Administration (TGA). The TGA is responsible for assessing the package of evidence collected after several years of research, to verify the claimed quality, safety and efficacy of the medicine. This process takes about one year and includes specialist evaluation and analysis of all data collected during the several years of investigation of the new treatment.

Once the TGA approves a medicine, it is recorded on the Australian Register for Therapeutic Goods (ARTG). At this point, the medicine is considered to have "marketing authorisation" and can legally be sold in Australia. The medicine is then subject to ongoing, continuous monitoring, known as pharmacovigilance, by the TGA and the manufacturer to ensure the continued quality, safety and efficacy of the medicine as it is more widely used.

The registration approval process also includes the review and approval by the TGA of Product Information (PI) and Consumer Medicines Information (CMI), as key documents to ensure the quality, safe and wise selection and appropriate use of medicines by the prescriber and the consumer.[3]

REGISTERING A MEDICINE
USUALLY TAKES 11 MONTHS

PBAC ASSESSMENT
USUALLY TAKES 4 MONTHS

PBS LISTING
TAKES A MINIMUM OF 4 MONTHS

R&D

1

Submit regulatory dossier to TGA

ACPM considers data on Quality, Safety and Efficacy

APPROVAL

Approval – Australian Register of Therapeutic Goods (ARTG)

3

SUBMIT PATIENT VIEWS

Consumers

5

HEARING

REJECT

RE-SUBMISSION

PARALLEL PROCESSING

NON-PARALLEL PROCESS

Submit to PBAC

2

Submit to PBAC

PBAC considers submission

Recommend

Price and listing negotiation

4

Approval by the minister (and if required cabinet)

PBS

PBS

ABOUT PBAC SUBMISSION

Types	Request
Major	New listing
Minor	Changes to current listings (e.g forms, restrictions)
Re-submission	Like a major submission
Secretariat/ PEB	Listing of generic medicines

ABOUT MEDICINES RESEARCH

Basic research & drug discovery. Preclinical trial on non - human subjects. Clinical trials on human subjects.

- Phase 0: Test how medicines are absorbed, distributed in the body, metabolised and eliminated from the body
- Phase 1: Trying to find the range of safe doses in healthy subjects
- Phase 2: Testing safety and tolerability (and efficacy) in patients
- Phase 3: Testing efficacy effectiveness and safety in patients

HOW PBAC CONSIDERS A SUBMISSION

Comparative assessment

Drug A — Equal — New Med = **Same Price**

Drug A — Better — New Med = **Higher/lower price corresponding to:**
- Relative UP or DOWN in health benefits
- Government's "ICER" threshold

Drug A — Worse — = **Evidence not certain**
- Managed Access Program (MAP)

Decision making criteria

- QUANTIFIABLE

Clinical

Need

Efficacy

Cost-Effectiveness

Budget Impact

- LESS QUANTIFIABLE

- Confidence in clinical evidence and modelling assumption
- Equity concerns
- Severity of conditions
- Public health concerns
- Quality use of medicines
- Suitability of the medicines being listed on the PBS

COMPONENTS OF THE PBS

Programs

- General schedule medicines
- The Highly Specialised Drugs Program
- The Efficient Funding of Chemotherapy program
- The Botulinum Toxin Program
- The Growth Hormone Program
- The IVF Program
- The Opiate Dependence Treatment Program
- [Life Saving Drugs Program]

Programs

- Formulary 1: Single brand, usually on patent
- Formulary 2: Multiple brands, usually off patent
- Combination drug list: on & off-patent components

Incremental cost - effectiveness ration or ICER

Differences in costs of A vs B
Differences in benefits (e.g. QALY) between A and B

Criteria for MAP

- High and urgent unmet clinical needs
- PBAC would NOT otherwise recommend the listing because of uncertainty or high cost
- Evidence can reliably be reported and evaluated within a reasonable timeframe (i.e. real world evidence).

Source – GSK/ViiV Healthcare

#2 Medicines Listing on the PBS

The medicine can now be considered for listing on the PBS. The manufacturer of the medicine needs to submit another package of clinical and economic evidence to the PBAC, to demonstrate the medicine's relative safety and efficacy and cost effectiveness compared to alternative treatments.

The PBAC consists of doctors, specialists, health professionals, health economists and consumer representatives, and is responsible for recommending to the Minister for Health which medicines are value for money and should be subsidised through the PBS at a cost-effective price.

Once the medicine is listed on the PBS there are further rules governing how the reimbursed, cost effective price can change, with statutory price reductions mandated at specific intervals and other reference comparisons made to the prices of similar listed medicines.

Note – Applications for PBS listing can be made in parallel to the application for ARTG registration, aimed at speeding up the time for medicine approval and reimbursement.

The Minister for Health and the Cabinet are gatekeepers to the PBS listing process. Positive PBAC recommendations need approval from the Minister (or their Delegate) to be listed, and medicines with cost over $20 million in any year of the forward estimates will also require Cabinet approval. The PBAC meets three times a year to consider submissions and convenes three additional inter-cycle meetings per year. Other ad-hoc meetings can occur throughout the year to discuss PBS-related matters.

As part of its assessment the PBAC will evaluate the new medicine to comparable medicines already available for the relevant condition, including a cost effectiveness analysis. The assessment is performed using five quantitative factors:

1. Comparative health gains

- Does the medicine offer patient outcomes that are significantly better than those offered by existing treatments?

2. Comparative cost-effectiveness

- Can the medicine be listed at a reasonable price compared to existing treatments?

3. Patient affordability

- Is a government subsidy necessary to achieve the aims of access and equity?

4. Predicted use and impact on PBS budget

5. Impact on health budget

The PBAC will also assess under less quantifiable factors including[4]:

- Confidence - Is there confidence in the clinical evidence and economic analysis?
- Effective alternative treatments - Is there a prevailing clinical need for a new treatment option?
- Equity concerns - Assessment of the age, socio-economic background and geographical status of those most likely to require treatment.

Other factors:

- Ability to target the target population.
- Public health concerns.
- Severity of the medical condition to be treated by the new medicine.
- Consumer input – including the views of effected consumers on the need, access and impact of a new therapy.

Once a medicine receives a positive recommendation from the PBAC, the Department of Health commences negotiations with the medicine manufacturer regarding pricing and, where required, a Risk Share Agreement (RSA). This is a formal Deed of Agreement that provides the contractual conditions of the medicine's availability and arrangements to manage the financial risk if the medicine is used outside the cost effective patient population, to avoid unexpected costs to the taxpayer. 80% of medicine manufacturers expect to use a Risk Sharing Agreement for new listings in the foreseeable future.[5]

If the medicine has equivalent health outcomes to others already available, the Department will set an equivalent price (cost minimisation). If the medicine is judged to be superior to an existing treatment, then the price will be set in relation to the compared level of effectiveness.

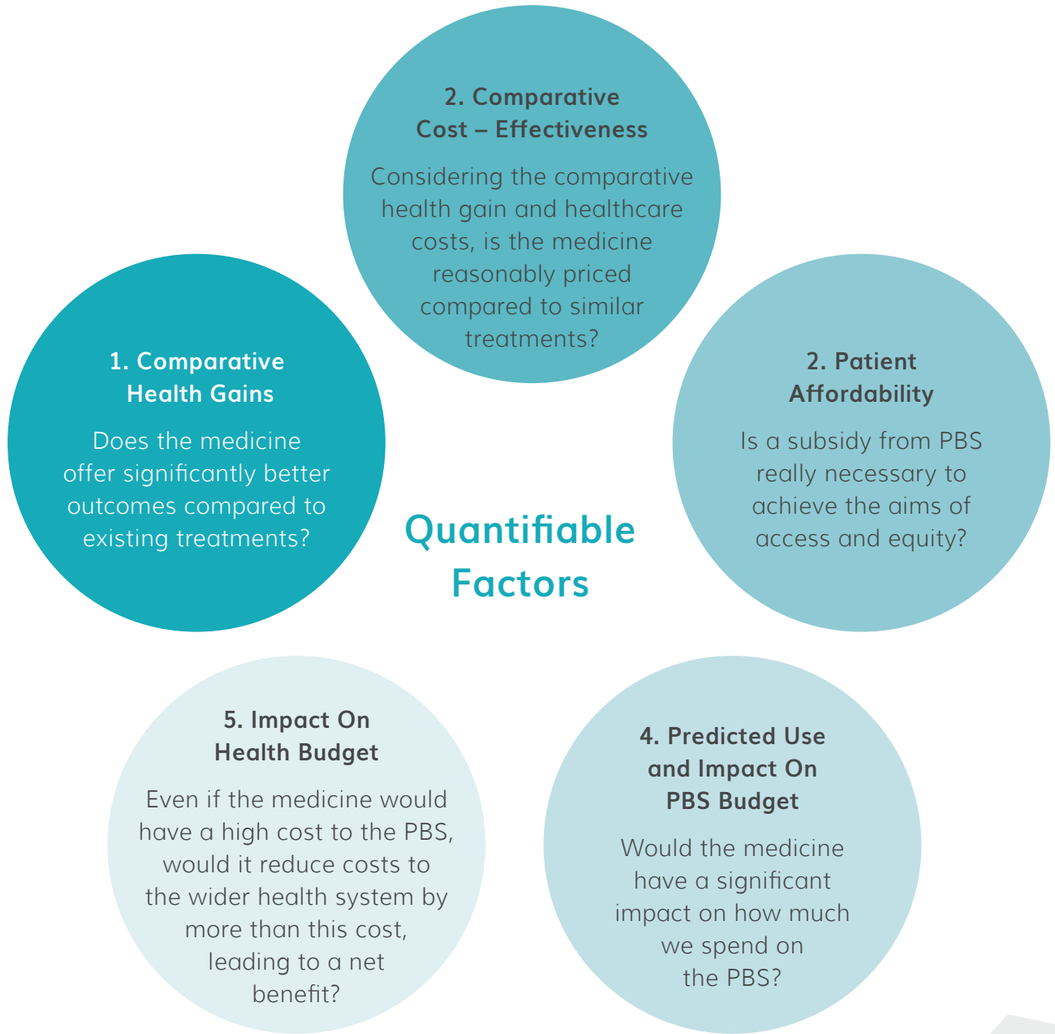
Australia has a tough assessment regime and represents a very small proportion of the global pharmaceutical market (1-2%). To ensure that medicines manufacturers can continue to bring innovative medicines to the Australian market, particularly when Australia commonly pays much lower prices for medicines compared to similar countries, the government has agreed to a process of special pricing arrangements (SPA). Under an SPA the manufacturer may offer a confidential discount on the price of the medicine. The published price, commonly higher than the confidential discounted price, will represent the indicative value of the medicine.

Actual medicine prices are negotiated as a bilateral agreement directly between the manufacturer and the Commonwealth Government. The difference between the confidential discounted price and the published price is repaid to the Australian Commonwealth Government through a rebate mechanism. Special Pricing Arrangements are crucial to the ability

of manufacturers to bring medicines to Australia, are critical to the maintenance of medicine access to Australian patients and provide significant benefit to the taxpayer.

More than two-thirds of Australians agree that the PBS and the innovative medicines industry delivers a more prosperous future for Australia.[6] Central to this is a strong collaboration between industry and government, and an understanding by policy makers of the long timeframes and large costs involved in medicine development.

Quantifiable Factors



Less Quantifiable Factors

1 http://www.pbs.gov.au/statistics/expenditure-prescriptions/2017-2018/expenditure-and-prescriptions-twelve-months-to-30-june-2018.pdf

2 Nielsen Research (2019), "Medicines Australia Consumer Sentiment Index". Available at www.medicinesaustralia.com.au

3 The Pharmaceutical Benefits Scheme in Australia; An Explainer on System Components, GSK. Available at: https://au.gsk.com/en-au/behind-the-science/how-we-do-business/pulling-back-the-curtain-on-the-pbs/

4 The Pharmaceutical Benefits Scheme in Australia; An Explainer on System Components, GSK. Available at: https://au.gsk.com/en-au/behind-the-science/how-we-do-business/pulling-back-the-curtain-on-the-pbs/

5 PwC (2015) "Challenges and Change: A Report on the Australian Pharmaceutical Industry", available at: https://www.pwc.com.au/industry/healthcare/assets/challengesa-and-changes-final.pdf

6 Nielsen Research (2019), "Medicines Australia Consumer Sentiment Index". Available at www.medicinesaustralia.com.au

Medicines Australia

Better health through research and innovation

www.medaus.com.au