PARLIAMENTARY INQUIRY QUESTION ON NOTICE

Department of Health

Standing Committee on Health, Aged Care and Sport

Approval processes for new drugs and novel medical technologies in Australia

18 June 2021

PDR Number: IQ21-000124

Details of PBAC e.g. Length of tenure, appointment of members, terms of reference, copy of legislation

Spoken

Hansard Page number: 31

Senator: Mike Freelander

Question:

Dr FREELANDER: Just a couple. They're more procedural things that I don't quite understand. With the PBAC, who makes up the PBAC and how long is their tenure and how are they chosen?

Ms Platona: It's legislated.

Dr FREELANDER: Yes.

Ms Platona: The legislation, the National Health Act, specifies that it's a committee of 21, with a chair.

Dr FREELANDER: Right.

Ms Platona: The legislation is also quite prescriptive about how to seek nominations and where to seek the nominations from. It's not for every member, but, for example the legislation prescribes that there needs to be a pharmacist, a general practitioner and a medical specialist. We can provide that on notice. The term is four years.

Dr FREELANDER: And they're appointed?

Ms Platona: They're appointed by the Minister for Health and the appointments are also at the discretion of the Prime Minister, subject to cabinet consideration. The PBAC appointments have in the past all gone for cabinet approval. The appointment term is four years or two years, depending on the mix of the committee's composition, to make sure that they don't all go at the same date and we have all brand-new members. There's a mix of new and old there, either for two years or four years. We can provide the exact words of the legislation to you about the membership of the PBAC, if you wish.

Dr FREELANDER: Just those details would be good.

Answer:

The composition and the functions of the Pharmaceutical Benefits Advisory Committee (PBAC) are set out in sections 100A to 101A of the *National Health Act 1953*. These sections are provided in full in **Attachment A** and cover:

- establishment and membership of the committee, including required specialities
- the nomination and appointment process
- termination of appointment
- remuneration
- the functions of the committee
- sub-committees.

Two-thirds of the members are selected from the interests/professions listed in paragraphs 100A(3) (a) to (f) and the remaining members are those who have relevant qualifications and experience as agreed by the Minister.

Nominating bodies are specified in section 86 of the National Health (Pharmaceutical Benefits) Regulations 2017 (see **Attachment A**). Nominations can also be sought from additional organisations.

Members are appointed for either two or four year terms depending on the vacancy to be filled, the level of expertise and experience of the nominee, and the availability of the nominee.

Once nominations are received, the Department in consultation with the Chair and Deputy Chair of the PBAC consider expertise, experience and potential conflicts of interest before making a recommendation to the Minister on preferred nominees.

The current membership of the committee is provided at **Attachment B**.

Attachment A

Sections 100A to 101A of the *National Health Act 1953*

100A Establishment and membership of the Pharmaceutical Benefits Advisory Committee

(1) There is to be a Committee called the Pharmaceutical Benefits Advisory Committee.

(2) The Committee is to consist of the Chairperson and at least 11, but not more than 20, other members.

Note: One of the members of the Committee (other than the Chairperson) maybe appointed as the Deputy Chairperson of the Committee: see subsection 100B(1D).

(3) Members forming at least 2/3 of the total membership of the Committee are to be selected from the following:

(aa) industry

(a) consumers

(b) health economists

(c) practising community pharmacists

(d) general practitioners

(e) clinical pharmacologists

(f) specialists.

with at least one member selected from each of the interests or professions mentioned in paragraphs (a) to (f).

(4) The remaining members (if any) of the Committee are to be persons whom the Minister is satisfied have qualifications or experience:

(a) in a field relevant to the functions of the Committee, and

(b) that would enable them to contribute meaningfully to the deliberations of the Committee.

(5) The Chairperson is a member of the Committee.

100B Appointment etc. of members of the Pharmaceutical Benefits Advisory Committee

(1) The members of the Pharmaceutical Benefits Advisory Committee are to be appointed by the Minister by written instrument.

(1AA) A person appointed under subsection 100A(3) in respect of paragraph 100A(3)(aa) must be appointed from nominations made by the following:

(a) industry organisations prescribed by the regulations for the purposes of this paragraph

(b) industry organisations that the Minister invites to make nominations for the appointment.

(1AB) A person appointed under subsection 100A(3) in respect of paragraph 100A(3)(a) must be appointed from nominations made by the following:

(a) consumer organisations prescribed by the regulations for the purposes of this paragraph

(b) individuals or consumer organisations that the Minister invites to make nominations for the appointment.

(1A) A person appointed under subsection 100A(3) must be appointed from nominations made by the following bodies:

(b) in respect of paragraph 100A(3)(b)—professional associations of health economists

(c) in respect of paragraph 100A(3)(c)—professional associations of pharmacists

(d) in respect of paragraph 100A(3)(d)—professional associations of medical practitioners

(e) in respect of paragraph 100A(3)(e)—professional associations of clinical pharmacologists

(f) in respect of paragraph 100A(3)(f)—professional associations of specialists prescribed by the regulations for the purposes of this subsection.

(1B) The regulations may prescribe matters relating to nominations, including (but not limited to) the number of nominations to be considered by the Minister before making an appointment.

(1C) The Minister must appoint one of the members of the Committee as the Chairperson of the Committee.

(1D) The Minister may appoint one of the members of the Committee (other than the Chairperson) as the Deputy Chairperson of the Committee.

(1E) The Chairperson and the Deputy Chairperson (if any) are to be appointed on a full-time or part-time basis.

(1F) The other members of the Committee are to be appointed on a part-time basis.

(2) A member of the Committee is eligible for reappointment.

(3) The performance of the functions and the exercise of the powers of the Committee are not affected merely because the number of members of the Committee falls below 12 for a period of not more than 6 months.

(4) The names and qualifications of the members of the Committee must be published in the *Gazette.*

100C Termination of appointment

A member of the Pharmaceutical Benefits Advisory Committee holds office during the Minister's pleasure.

Attachment A

100D Remuneration

(1) A member of the Pharmaceutical Benefits Advisory Committee is to be paid the remuneration that is determined by the Remuneration Tribunal. If no determination of that remuneration by the Tribunal is in operation, the member is to be paid the remuneration that is prescribed.

(2) A member is to be paid the allowances that are prescribed.

(3) This section has effect subject to the *Remuneration Tribunal Act 1973*.

101 Functions of Pharmaceutical Benefits Advisory Committee

Functions relating to drugs and medicinal preparations

(3) The Pharmaceutical Benefits Advisory Committee shall make recommendations to the Minister from time to time as to the drugs and medicinal preparations which it considers should be made available as pharmaceutical benefits under this Part and shall advise the Minister upon any other matter concerning the operation of this Part referred to it by the Minister.

(3AA) The Pharmaceutical Benefits Advisory Committee must make recommendations to the Minister from time to time about what should be specified in a determination under subsection 84AAA(2).

(3AB) Subsection (3AA) does not limit subsection (3).

(3A) For the purpose of deciding whether to recommend to the Minister that a drug or medicinal preparation, or a class of drugs and medicinal preparations, be made available as pharmaceutical benefits under this Part, the Committee shall give consideration to the effectiveness and cost of therapy involving the use of the drug, preparation or class, including by comparing the effectiveness and cost of that therapy with that of alternative therapies, whether or not involving the use of other drugs or preparations.

(3B) Without limiting the generality of subsection (3A), where therapy involving the use of a particular drug or medicinal preparation, or a class of drugs and medicinal preparations, is substantially more costly than an alternative therapy or alternative therapies, whether or not involving the use of other drugs or preparations, the Committee:

(a) shall not recommend to the Minister that the drug, preparation or class be made available as pharmaceutical benefits under this Part unless the Committee is satisfied that the first-mentioned therapy, for some patients, provides a significant improvement in efficacy or reduction of toxicity over the alternative therapy or therapies, and

(b) if the Committee does recommend to the Minister that the drug, preparation or class be made available as pharmaceutical benefits under this Part, the Committee shall include in its recommendation a statement that the Committee is satisfied as mentioned in paragraph (a).

(3BA) If the Committee is of the opinion that a drug or medicinal preparation should be made available as a pharmaceutical benefit under this Part, the Committee must, in its recommendation under subsection (3), specify whether the drug or medicinal preparation and another drug or medicinal preparation should be treated as interchangeable on an individual patient basis.

(3C) Where the Committee is of the opinion that a drug or medicinal preparation, or a class of drugs and medicinal preparations, should be made available as pharmaceutical benefits under this Part, but only in certain circumstances, the Committee shall, in its recommendation under subsection (3), specify those circumstances.

Functions relating to declarations under subsection 85(2)

(4) A drug or medicinal preparation shall not be declared, pursuant to paragraph 85(2)(a), to be a drug or medicinal preparation in relation to which this Part applies unless:

(a) the drug or medicinal preparation was, immediately before the commencement of this subsection, a pharmaceutical benefit; or

(b) the Committee has recommended to the Minister that it be so declared.

(4A) A class of drugs or medicinal preparations, or of drugs and medicinal preparations, shall not be declared, pursuant to paragraph 85(2)(a), to be a class of drugs or medicinal preparations, or of drugs and medicinal preparations, in relation to which this Part applies unless:

(a) each member of that class was, immediately before the commencement of this subsection, a pharmaceutical benefit; or

(b) the Committee has recommended to the Minister that the class be so declared.

(4AAA) The Minister may, by legislative instrument, revoke or vary a declaration under subsection 85(2) in relation to a drug or medicinal preparation.

(4AAB) If:

(a) under subsection (4AAA), the Minister proposes to revoke or vary a declaration under subsection 85(2) in relation to a drug or medicinal preparation

(b) on and after the day the revocation or variation comes into force, the drug or medicinal preparation would cease to be a listed drug

then, before making the revocation or variation, the Minister must obtain the advice in writing of the Pharmaceutical Benefits Advisory Committee in relation to the proposed revocation or variation.

(4AAC) An advice under subsection (4AAB) must be laid before each House of the Parliament with the declaration under subsection (4AAA) to which the advice relates.

Functions relating to determinations under section 88

(4AACAA) The Pharmaceutical Benefits Advisory Committee may give advice to the Minister as to which PBS prescribers should be authorised to write prescriptions for the supply of a pharmaceutical benefit.

Functions relating to declarations under subsection 85(2AA)

(4AACA) The Pharmaceutical Benefits Advisory Committee must make recommendations to the Minister from time to time as to the drugs and medicinal preparations which it considers should only be supplied under one or more of the prescriber bag provisions.

(4AACB) The Minister may, by legislative instrument, revoke or vary a declaration under subsection 85(2AA) in relation to a drug or medicinal preparation.

(4AACC) If:

(a) under subsection (4AACB), the Minister proposes to revoke or vary a declaration under subsection 85(2AA) declaring that a drug or medicinal preparation (the ***drug***) can only be supplied under one or more of the prescriber bag provisions

(b) on and after the day the revocation or variation comes into force, the drug could be supplied under this Part otherwise than under one or more of the prescriber bag provisions

then the Minister can only make the revocation or variation if:

(c) the Minister also revokes or varies the declaration under subsection 85(2), in accordance with subsections (4AAA), (4AAB) and (4AAC) of this section, so that the drug ceases to be a listed drug on and after the day the revocation or variation of the subsection 85(2) declaration comes into force, or

(d) the Pharmaceutical Benefits Advisory Committee recommends against the Minister taking the action in paragraph (c).

Functions relating to determinations under subsection 85(6A)

(4AACD) The Pharmaceutical Benefits Advisory Committee may give advice to the Minister in relation to whether or not the Minister should determine that a brand of a pharmaceutical item is to be treated as equivalent to one or more other brands of pharmaceutical items.

Functions relating to declarations under subsection 85(2A)

(4AAD) The Pharmaceutical Benefits Advisory Committee must make recommendations to the Minister from time to time as to the drugs and medicinal preparations which it considers should be made available only under special arrangements under section 100.

(4AAE) The Minister may, by legislative instrument, revoke or vary a declaration under subsection 85(2A) in relation to a drug or medicinal preparation.

(4AAF) If:

(a) under subsection (4AAE), the Minister proposes to revoke or vary a declaration under subsection 85(2A) in relation to a drug or medicinal preparation (the ***drug***)

(b) on and after the day the revocation or variation comes into force, the drug could be supplied under this Part otherwise than under special arrangements under section 100

then the Minister can only make the revocation or variation if:

(c) the Minister also revokes or varies the declaration under subsection 85(2), in accordance with subsections (4AAA), (4AAB) and (4AAC) of this section, so that the drug ceases to be a listed drug on and after the day the revocation or variation of the subsection 85(2) declaration comes into force, or

(d) the Pharmaceutical Benefits Advisory Committee recommends against the Minister taking the action in paragraph (c).

Function relating to Minister's determination of therapeutic groups

(4AA) If the Committee is of the opinion that the Minister should, or should not, determine a therapeutic group, the Committee must advise the Minister accordingly.

Function relating to Minister's determination about exempt items

(4AB) If the Committee is of the opinion that the following circumstances exist in relation to a pharmaceutical item:

(a) the listed drug in the pharmaceutical item represents suitable therapy for a particular patient population

(b) the pharmaceutical item is suitable for use by a particular subgroup of that population because of either or both of the form and manner of administration of the drug in the item

(c) no other pharmaceutical item that has that drug is suitable for use by that subgroup because of either or both of the form and manner of administration of the drug in that other item

the Committee must advise the Minister that those circumstances exist in relation to the pharmaceutical item.

Function relating to Minister's decisions about prices of combination items

(4AC) If the Committee is satisfied that therapy involving a combination item provides, for some patients:

(a) a significant improvement in patient compliance with the therapy, or

(b) a significant improvement in efficacy or reduction in toxicity

over alternative therapies, then the Committee must advise the Minister accordingly.

Functions relating to determinations that brands are not new brands

(4AD) The Pharmaceutical Benefits Advisory Committee may give advice to the Minister in relation to whether the Minister should determine that a brand of a pharmaceutical item is not a new brand for the purposes of section 99ACB or 99ACD.

Functions relating to vaccines

(4B) The Pharmaceutical Benefits Advisory Committee must:

(a) make recommendations to the Minister from time to time about the vaccines it considers should be designated vaccines (see section 9B), and

(b) advise the Minister about any other matter concerning the operation of section 9B referred to it by the Minister.

(4C) For the purpose of deciding whether to recommend to the Minister that a vaccine be a designated vaccine, the Committee must give consideration to the effectiveness and cost of immunisation involving the use of the vaccine, including by comparing the effectiveness and cost of immunisation involving the use of the vaccine with the effectiveness and cost of alternative options, whether or not involving the use of other vaccines.

(4D) If immunisation involving the use of a particular vaccine (the ***first vaccine***) is substantially more costly than an alternative vaccine:

(a) the Committee must not recommend to the Minister that the first vaccine be a designated vaccine unless the Committee is satisfied that the first vaccine, for some individuals, provides a significant improvement in efficacy or reduction of toxicity over the alternative vaccine, and

(b) if the Committee recommends to the Minister that the first vaccine be a designated vaccine—the Committee must include in its recommendation a statement that the Committee is satisfied as mentioned in paragraph (a).

(4E) Subsection (4D) does not limit subsection (4C).

(4F) If the Committee is of the opinion that a vaccine should be a designated vaccine, but should only be provided under subsection 9B(1) in certain circumstances, the Committee must, in its recommendation under subsection (4B), specify those circumstances.

Procedure

(5) The regulations may make provision for and in relation to the procedure of the Committee.

101A Sub-committees of the Pharmaceutical Benefits Advisory Committee

(1) The Pharmaceutical Benefits Advisory Committee:

(a) may establish such sub-committees as it thinks fit to assist it in performing its functions, and

(b) shall, if the Minister so requires in writing, establish a sub-committee to assist the Committee in advising the Minister on a particular matter referred to it by the Minister under subsection 101(3) or (4B).

(2) A sub-committee shall consist of the following persons (whether or not members of the Committee):

(a) persons appointed by the Committee as members of the sub-committee

(b) persons nominated by the Minister as members of the sub-committee.

(3) A person shall not be appointed by the Committee, or nominated by the Minister, as a member of a sub-committee unless the person has special qualifications or experience in relation to the matter referred to the sub-committee.

(4) For the purposes of section 140, a sub-committee shall be taken to be a committee established under this Act.

Attachment A

National Health (Pharmaceutical Benefits) Regulations 2017

Part 8—Arrangements for the Pharmaceutical Benefits Advisory Committee

Division 1—Matters relating to the appointment of members of the Committee

86 Nominating bodies

Industry organisations

(1) For the purposes of paragraph 100B(1AA)(a) of the Act, the following industry organisations are prescribed:

(a) Medicines Australia Limited

(b) Generic Medicines Industry Association Pty Ltd trading as the Generic and Biosimilar Medicines Association

(c) Ausbiotech Ltd.

Consumer organisations

(2) For the purposes of paragraph 100B(1AB)(a) of the Act, the following consumer organisations are prescribed:

(a) the Consumers Health Forum of Australia Ltd

(b) the Australian Federation of AIDS Organisations Incorporated

(c) the Australian Consumers' Association.

Professional associations

(3) For the purposes of paragraph 100B(1A)(b) of the Act, the following professional associations of health economists are prescribed:

(a) the Australian Health Economics Society Inc

(b) the Economic Society of Australia Inc.

(4) For the purposes of paragraph 100B(1A)(c) of the Act, the following professional associations of pharmacists are prescribed:

(a) the Pharmacy Guild of Australia

(b) the Pharmaceutical Society of Australia

(c) the Society of Hospital Pharmacists of Australia.

(5) For the purposes of paragraph 100B(1A)(d) of the Act, the following professional associations of medical practitioners are prescribed:

(a) the Australian Medical Association Limited

(b) the Royal Australian College of General Practitioners

(c) the Doctors Reform Society—Australia Inc

(d) the Australian Federation of Medical Women Inc.

(6) For the purposes of paragraph 100B(1A)(e) of the Act, the following professional associations of clinical pharmacologists are prescribed:

(a) the Royal Australasian College of Physicians

(b) the Australasian Society of Clinical and Experimental Pharmacologists and Toxicologists.

(7) For the purposes of paragraph 100B(1A)(f) of the Act, the following professional associations of specialists are prescribed:

(a) the Australian Medical Association Limited

(b) the Royal Australasian College of Physicians

(c) the Committee of Presidents of Medical Colleges.

87 Number of nominations for appointment

For the purposes of subsection 100B(1B) of the Act, each body prescribed for the purposes of subsection 100B(1AA), (1AB) or (1A) of the Act must be asked to nominate at least 3 persons for selection for appointment as members of the Pharmaceutical Benefits Advisory Committee.

88 Resignation

A member of the Pharmaceutical Benefits Advisory Committee may resign by notice in writing given to the Minister.

Division 2—Matters relating to the procedure of the Committee

89 Purpose of this Division

For the purposes of subsection 101(5) of the Act, this Division makes provision for and in relation to the procedure of the Pharmaceutical Benefits Advisory Committee.

90 Presiding member

(1) The Chairperson of the Pharmaceutical Benefits Advisory Committee must preside at a meeting of the Committee if the Chairperson is present.

(2) If the Chairperson is absent and there is a Deputy Chairperson of the Committee present, the Deputy Chairperson must preside at the meeting.

(3) If:

(a) the Chairperson is absent, and

(b) there is no Deputy Chairperson present

the members of the Committee attending the meeting must elect a member to preside at the meeting.

91 Meetings of the Committee

(1) The Chairperson of the Pharmaceutical Benefits Advisory Committee may, at any time, by notice in writing to all members of the Committee, convene a meeting of the Committee.

(2) The Committee must keep minutes of its meetings.

92 Quorum

At a meeting of the Pharmaceutical Benefits Advisory Committee, a quorum is the number of members who constitute a majority of the membership of the Committee.

93 Voting

(1) At a meeting of the Pharmaceutical Benefits Advisory Committee, the members present each have a deliberative vote.

(2) A matter requiring a decision at a meeting must be determined by a majority of the votes of the members present and voting.

(3) If an equal number of votes is cast for and against a matter at a meeting:

(a) the member presiding at the meeting may exercise a casting vote, and

(b) if that member declines to exercise a casting vote—the matter is resolved in the negative.

(4) Decisions of the Committee must be recorded in the minutes of the meeting.

94 Disclosure of pecuniary interests by members

(1) Each member of the Pharmaceutical Benefits Advisory Committee must tell the Minister in writing, as soon as practicable after the beginning of each financial year, of all direct or indirect pecuniary interests that the member has, or proposes to acquire, in a business or in a body corporate carrying on a business that could conflict with the member's duties.

(2) If a member does not have an interest of the kind referred to in subsection (1), the member must give a statement to that effect to the Minister.

(3) If the member presiding at a meeting of the Committee has a direct or indirect pecuniary interest in a matter that is to be considered at the meeting, the presiding member:

(a) must disclose the interest to the other members present at the meeting, and

(b) must not take part in the meeting during the consideration of that matter unless the other members present at the meeting agree that the presiding member may take part in the meeting.

(4) If the presiding member is precluded from taking part in a meeting or part of a meeting because of paragraph (3)(b):

(a) if the presiding member is the Chairperson of the Committee and a Deputy Chairperson of the Committee is present—the Deputy Chairperson must act in the place of the Chairperson for the duration of the Committee's consideration of the matter; or

(b) if:

(i) the presiding member is the Chairperson of the Committee and no Deputy Chairperson of the Committee is present, or

(ii) the Deputy Chairperson is the presiding member

the other members attending the meeting must elect a member who is present to act in the place of the presiding member for the duration of the Committee's consideration of the matter.

(5) If a member (other than the presiding member) of the Committee has a direct or indirect pecuniary interest in a matter that is to be considered at a meeting of the Committee, the member:

(a) must disclose the interest to the presiding member at the commencement of the meeting, and

(b) must not take part in the meeting during the consideration of that matter unless the presiding member allows the member to take part in the meeting.

(6) The following matters must be recorded in the minutes of a meeting of the Committee:

(a) a disclosure made under subsection (3) or (5)

(b) an agreement under paragraph (3)(b)

(c) consent of the presiding member under paragraph (5)(b).

95 Resolutions without a formal meeting

If a majority of the members of the Pharmaceutical Benefits Advisory Committee sign a document that includes a statement that they are in favour of a resolution in the terms set out in the document, the resolution is taken to have been passed at a meeting of the Committee:

(a) on the day on which the document is signed, or

(b) if the members sign the document on different days—on the day on which the document is signed by the member who completes the majority.

96 Reports and recommendations

(1) A report or a recommendation made to the Minister by the Pharmaceutical Benefits Advisory Committee as part of its consideration of a matter must be in writing.

(2) If:

(a) the members of the Committee are not unanimous in agreeing to a report or a recommendation, and

(b) a member who is not part of the majority asks the Chairperson of the Committee to include, as part of the report or recommendation:

(i) a statement that the members are not unanimous

(ii) an explanation of the opinion of the member

(iii) a separate report or recommendation made by the member

the report or recommendation must include the matter requested by the member.

Division 3—Matters relating to sub-committees

97 Remuneration for chair and members of sub-committees

Fees and allowances payable to chairs

(1) For the purposes of paragraph 140(a) of the Act, the fees and allowances payable to the Chair of the Drug Utilisation Sub-Committee and the Chair of the Economics Sub-Committee are the amounts payable to the Chairperson of the Pharmaceutical Services Federal Committee of Inquiry in accordance with a determination made by the Remuneration Tribunal.

Fees and allowances payable to other members

(2) For the purposes of paragraph 140(a) of the Act, the fees and allowances payable to a member (other than the Chair) of the Drug Utilisation Sub-Committee or the Economics Sub-Committee are the amounts payable to a member (other than the Chairperson) of the Pharmaceutical Services Federal Committee of Inquiry in accordance with a determination made by the Remuneration Tribunal.

Definitions

(3) In this section:

Drug Utilisation Sub-Committee means the sub-committee of that name established under section 101A of the Act.

Economics Sub-Committee means the sub-committee of that name established under section 101A of the Act.

Attachment B

Current PBAC Membership list (2 July 2021)

Position	Appointee	Start Date	End Date
Chair	Prof Andrew Wilson	5 May 2019	4 May 2023
Deputy Chair	Ms Jo Watson	8 May 2019	7 May 2023
Member	Prof Chris Etherton-Beer	25 June 2020	24 June 2022
Member	Dr Ray Parkin	25 June 2020	24 June 2024
Member	Dr Shane Hamblin	25 July 2017	24 July 2021
Member	Dr Peggy Brown	25 June 2020	24 June 2022
Member	Prof Jonathan Craig	6 May 2019	5 May 2023
Member	Ms Michelle Burke	4 July 2019	3 July 2023
Member	Prof Phoebe Joy Ho	25 July 2017	24 July 2021
Member	A/Prof Peter Grimison	5 May 2019	4 May 2023
Member	Dr Thomas Snelling	20 February 2019	19 February 2023
Member	Dr Elizabeth Marles	20 February 2019	19 February 2023
Member	Prof Clement Loy	20 February 2019	19 February 2023
Member	Dr Kylie Mason	20 February 2019	19 February 2023
Member	Dr Meena Okera	25 June 2020	24 June 2024
Member	Prof Kirsten Howard	30 June 2021	29 June 2025
Member	Prof Catherine Hill	30 June 2021	29 June 2023
Member	Dr Jo- Anne Manski-Nankervis	6 May 2019	5 May 2023
Member	Dr David Newby	30 June 2021	29 June 2025
Member	Dr Peter Fox	30 June 2021	29 June 2025
Member	Dr Susannah Morris	30 June 2021	29 June 2025

PARLIAMENTARY INQUIRY QUESTION ON NOTICE

Department of Health

Standing Committee on Health, Aged Care and Sport

Inquiry into approval processes for new drugs and novel medical technologies in Australia

18 June 2021

PDR Number: IQ21-000125

Life-saving drugs program – criteria, flow chart of product journey

Spoken

Hansard Page number: 31

Senator: Mike Freelander

Question:

Dr FREELANDER: There are some questions that the secretariat would like in writing, so we'll put them on notice—if you don't mind, that would be good. The other very quick question is about the Life Saving Drugs Program. I'm not quite sure how the criteria are decided for which drugs can go on that pathway and how that decision is made.

Ms Platona: The eligibility criteria are government decisions.

Dr FREELANDER: It was a government introduction, wasn't it?

Ms Platona: Correct. There was a review in 2014-15 and a subsequent government consideration about introduction of new criteria. We'll be very happy to share that with you. If you'd like a description of the flow of the journey of the product from PBAC and PBS rejection onto LSDP, we'd be happy to provide that.

Dr FREELANDER: Thank you.

Answer:

Before a medicine is considered for inclusion on the Life Saving Drugs Program (LSDP), a medicine must first be considered by the Pharmaceutical Benefits Advisory Committee (PBAC) and accepted as clinically effective but rejected for Pharmaceutical Benefits Scheme (PBS) listing because it fails to meet the required cost-effectiveness criteria.

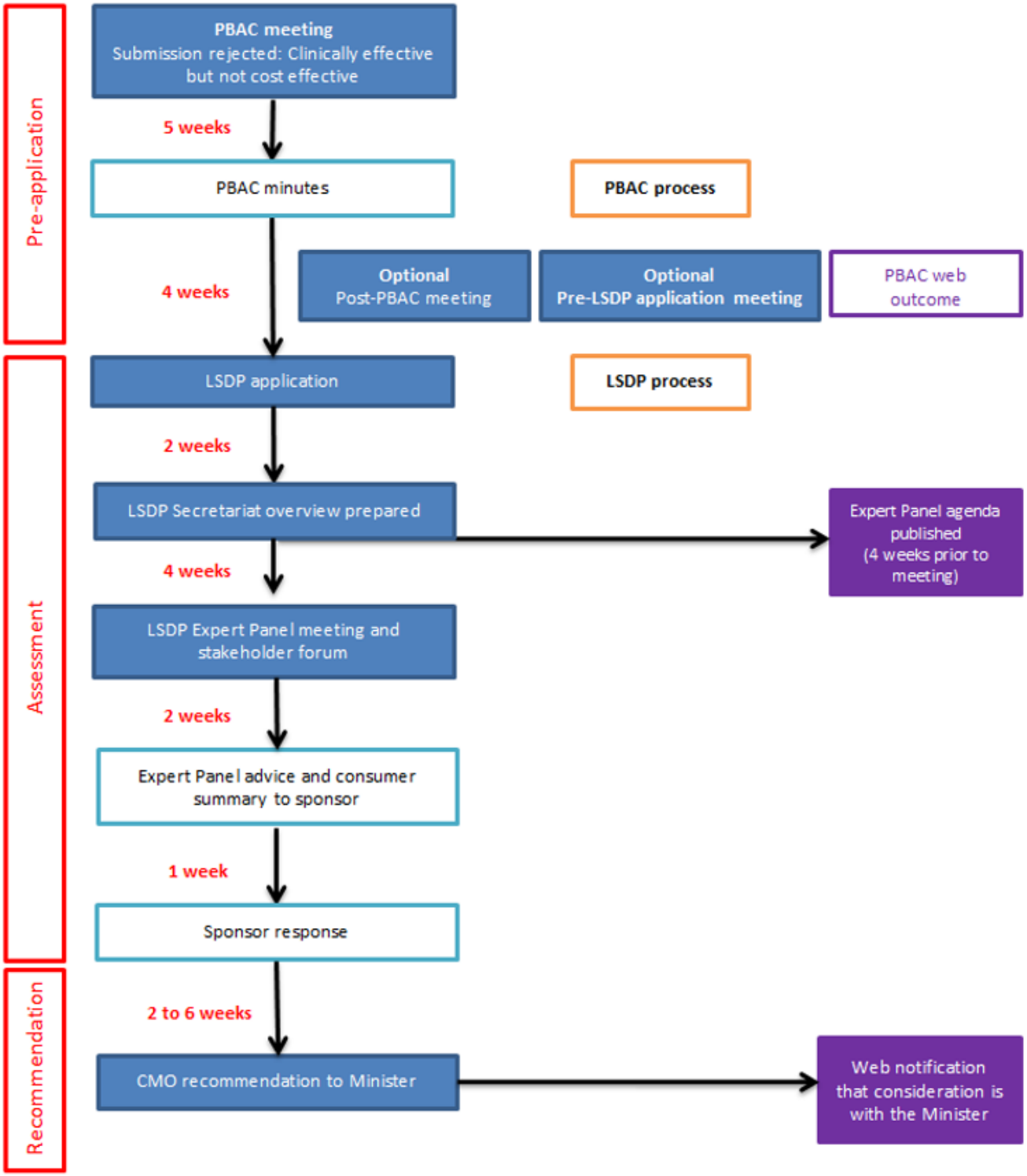
The procedure for consideration and listing of new medicines for subsidy through the LSDP is at Attachment A.

The *Procedure guidance for medicines funded through the LSDP* outlines the agreement in May 2018 between Government and Medicines Australia (on behalf of sponsors) to provide policy stability, transparency and certainty for the rare diseases medicine sector. This document includes the criteria for assessment and inclusion of medicines funded through the LSDP (Attachment B) and is available to the public on the Department's website at:

www.health.gov.au/initiatives-and-programs/life-saving-drugs-program.

Attachment A

PROCEDURE FOR CONSIDERATION OF NEW MEDICINES FOR SUBSIDY THROUGH THE LSDP



Attachment B

LSDP medicine inclusion Criteria (*excerpt Procedure guidance for medicines funded through the Life Saving Drugs Program* v1.0 July 2018)

Criterion A1.

There is a rare but clinically definable disease for which the drug is regarded as a proven therapeutic modality, i.e. approved for that indication by the Therapeutic Goods Administration.

Criterion A2.

The disease is identifiable with reasonable diagnostic precision.

Criterion A3.

Epidemiological and other studies provide evidence that the disease causes a significant reduction in age-specific life expectancy for those suffering from the disease.

Criterion A4.

There is evidence to predict that a patient's lifespan will be substantially extended as a direct consequence of the use of the drug.

Criterion A5.

The drug must be accepted as clinically effective, but rejected for Pharmaceutical Benefits Scheme (PBS) listing because it fails to meet the required cost effectiveness criteria.

Criterion A6.

There is no alternative drug listed on the PBS or available for public hospital in-patients, which can be used as lifesaving treatment for the disease. However, the availability of an alternative drug under the LSDP does not disqualify the proposed drug from consideration for the LSDP.

Criterion A7.

There is no alternative non-drug therapeutic modality (e.g. surgery, radiotherapy) which is recognised by medical authorities as a suitable and cost effective treatment for this condition.

Criterion A8.

The cost of the drug, defined as the cost per dose multiplied by the expected number of doses in a one year period for the patient, would constitute an unreasonable financial burden on the patient or his/her guardian.

Criterion B1.

The proposed confidential price of the drug compared with the effective price of the drug in comparable overseas markets.

Criterion B2.

The proposed cost of the drug compared with the cost of comparable drugs, if any, that are already funded through the LSDP.

PARLIAMENTARY INQUIRY QUESTION ON NOTICE

Department of Health

Standing Committee on Health, Aged Care and Sport

Inquiry into approval processes for new drugs and novel medical technologies in Australia

18 June 2021

PDR Number: IQ21-000126

Approval of a drug or a technology that had been approved by the EMA or the FDA

Spoken

Hansard Page number: 20

Member: Trent Zimmerman

Question:

CHAIR: Dr Skerritt, the issue of rubber stamping—well, there are two issues. One, I suppose, is there are drugs and technologies which different jurisdictions take entirely different decisions on—that is, approve or not approve. Then there's the more granular issue of decisions that might go to the application of those drugs or therapies to particular cohorts in the population, conditions of dispensing, all that type of thing. Is it that frequent that you would not approve a drug or a technology that had been approved by the EMA or the FDA? Dr Skerritt: It's uncommon for us not to approve a drug that's been approved by one or other, but there are examples. If need be, I can take it on notice and come back with specific ones.

Answer:

A key reason for the Governments decision in 2016 that Australia should continue to make sovereign decisions regarding medicines approvals, rather than 'rubber stamp' decisions of other regulators, was that there was often significant discordance between these decisions. In individual cases, this is thought to be due to differences between regulators in the data submitted by the applicant, differences in clinical practice or risk appetite between countries or differences in opinions between respective advisory committees. There have been some cases where absolute differences in regulatory outcome (acceptance versus rejection) occurred but much more common are significant differences in the approved indication (intended use) between regulators for a given medicine.

The differences in regulatory decisions between major regulators have been analysed in a number of reports in the refereed medical literature. Some of these studies are summarised below:

- Of 134 new drugs approved by all of US Food and Drug Administration (FDA), European Medicines Agency (EMA) and Swissmedic between 2007 and 2016, over three quarters (77 per cent) of the indications differed. In particular there were differences between US and European decisions (Zeukeng M-J et al. Eur J Clin Pharmacol. 74 (2018) 811).
- Of 115 new drugs approved by EMA or FDA or both between 2014-2016, there was a significant lack of concordance in approved indications (Kuhler T DC et al. BMJ Open 9: e028677 (2019)).
- For 107 applications to both FDA and EMA between 2014 and 2016, in eight cases FDA declined to approve the new drug in an initial application while EMA approved it, and in one case EMA rejected the initial application but FDA approved it (Kashoki M et al. Clin Pharm Ther 107 (2020) 195).
- Comparing 255 submissions between 2005 and 2014, rejection rates of for Swissmedic (16 per cent) were higher than for FDA (13 per cent) and the EMA (nine per cent), (Di Sanguinetto SDT et al. Therap Innov Reg Sci 53 (2019) 86).
- Of 80 new drugs first approved in Japan from 2008-2019, there were significant delays in Europe or the US for a number of drugs due to differences in assessment of clinical trials, differing regulatory reviews on cardiovascular risk and on regulatory processes between the three regions (Tanaka, M et al. Brit J Clin Pharmacol): https://bpspubs.onlinelibrary.wiley.com/doi/abs/10.1111/bcp.14749

PARLIAMENTARY INQUIRY QUESTION ON NOTICE

Department of Health

Standing Committee on Health, Aged Care and Sport

Inquiry into approval processes for new drugs and novel medical technologies in Australia

18 June 2021

PDR Number: IQ21-000127

FDA report on devices that they did not approve that were approved in Europe

Spoken

Hansard Page number: 20

Member: Trent Zimmerman

Question:

CHAIR: And technologies?

Dr Skerritt: I'll go to medical devices in a minute, and maybe my colleague Tracey Duffy will want to talk a bit more about it. Where there's a significant difference is actually in either the indications for a medicine or the intended use for a device. For example, the Europeans may say, 'This drug is for treatment of condition X,' whereas the Americans may come and say, 'This drug is for treatment of condition X when drug A has failed and also in adults over 25.' Where our advisory committees and our decision-making delegates spend a lot of time is looking at evidence for particular groups and also risk-benefit, because in some groups there might be a negative safety signal. For example, for a drug that you would approve for treatment of a particular cancer in someone in their 70s, the risk-benefit equation is very different from if that drug were to be used, say, in a gynaecological cancer that a woman in her 20s might have. It may affect a whole lot of things, including reproductive potential and so forth. What is very common is that the major regulators have different indications.

There's also a difference in law when it comes to medical devices. In Australia, devices are approved for an intended purpose—in other words, it says, for example, 'This device will renally denervate'—whereas some regulators, such as the FDA, will actually go to a different state and actually show a potential clinical benefit for a device. Now, that might sound like hair-splitting, and in many cases it is, but it takes you to a different place where we may approve a device because it chops off nerves, whereas they will approve a device because of a change in blood pressure, a clinical end point. So there is a difference in the detail.

I still am surprised that it didn't—well, I believe it did, actually—cause high-level diplomatic ruckus. It's not now 2012, but it was quite a significant document at the time. The FDA actually put out a report highlighting eight or nine devices that they did not approve that were approved in Europe. That report had quite an inflammatory title. Again, I'm happy to provide a copy of that report on notice. It is available on the internet. Basically, it ran along the lines of, 'These are devices that FDA has decided not to approve because of lower regulatory standards in Europe for medical devices.' The Europeans have totally overhauled their device regulatory system, and on 26 May this year, with a four-year transition period, it has come into play. That will require high levels of clinical evidence and standards. But for medical devices in particular, there's been a long-held view—and I think there is some truth in it—that European standards were not at the same high level as the US standards for medical device approvals.

In Australia, for many cases where a European approval was given, we did what's known as an application audit, where we further checked the clinical and other evidence. I think it's fair to say there have been a number of cases where it hasn't really met the requirements. So this was another issue behind the reluctance of government to support rubber stamping.

Answer:

The report is titled *'US FDA - Unsafe and Ineffective Devices Approved in the EU that were Not Approved in the US - May 2021'.*

A copy of the report is attached.

PARLIAMENTARY INQUIRY QUESTION ON NOTICE

Department of Health

Standing Committee on Health, Aged Care and Sport

Inquiry into approval processes for new drugs and novel medical technologies in Australia

18 June 2021

PDR Number: IQ21-000128

Countries with schemes to help medical start-up companies

Spoken

Hansard Page number: 22

Member: Trent Zimmerman

Question:

Dr Skerritt: What some governments have done is look at things like vouchers. In the same way we have an R&D tax concession and now we've got the patent box lower tax rate, it would be open to government to say, 'If you're a startup here, why not get a voucher for your first couple of products?' That's a decision by government, of course.

CHAIR: Off the top of your head, are there a couple of countries you can mention that have got such a scheme, or can you take it on notice?

Dr Skerritt: I'll take it on notice. They change all the time. In the US there have been various schemes. The Japanese have had schemes, but they keep on changing them, because this is an area of industry policy where they try something for a couple of years and it doesn't work and they change it. But I'm more than happy to take that on notice.

Answer:

Therapeutic Goods Administration (TGA) fees and charges for particular services are the same for small and large businesses, as the extent of work required to be performed by the TGA e.g. in evaluating a new medicine or medical device does not typically differ, depending on the size of the business. Indeed because there are often not dedicated regulatory affairs staff within small businesses, more iterations of review may be required by the TGA with a number of submission from small businesses.

If a decision by government to charge differential fees and charges for small businesses is made, changes to the *Therapeutic Goods (Charges) Act* would be required as well as a decision on how work that is no longer fully cost recovered would be funded. It is not permitted under Government Cost Recovery Guidelines to impose additional fees and charges on large businesses for the purposes of cross-subsidising small businesses.

While the list below is by no means exhaustive, major European and North American Regulators have a number of fees and charges waiver schemes for small businesses, with the shortfall in funding being made up through government appropriations. The criteria defining 'small' for businesses varies between regulators and particular waivers – for example the United States Food and Drug Administration (FDA) device fee reductions are available for businesses with annual turnovers as high as USD $100 million.

With the United States Food and Drug Administration (see: www.fda.gov/medical-devices/premarket-submissions/reduced-medical-device-user-fees-small-business-determination-sbd-program; www.fda.gov/media/72340/download), medical devices application types eligible for a reduced small business fees include a range of regulatory submissions and variations to submissions as well as some pre-market advice fees. There are some full fee waivers available for many small businesses for their first medical device, drug or biological product submission.

In Canada (see: www.canada.ca/en/health-canada/services/drugs-health-products/funding-fees/small-business-mitigation.html) a waiver is available to small businesses for their first ever medicines submission and a 50 per cent reduction for other evaluation fees. There is also a 25 per cent reduction for other fees, such as those for manufacturing establishment licences.

The European Medicines Agency (see: www.ema.europa.eu/en/human-regulatory/overview/fees-payable-european-medicines-agency) provide fee exemptions and reductions for pre- and post-authorisation regulatory procedures, including a 90 per cent reduction for scientific advice at: www.ema.europa.eu/en/glossary/scientific-advice, variations and inspections and pharmacovigilance. Fees for new medicines applications are also deferred until the outcome of the review is known.

The UK Medicines and Health products Regulatory Agency at: www.gov.uk/government/publications/mhra-fees/payment-easements-and-waivers-for-small-and-medium-companies defers invoicing of part of the fee for small businesses until a decision is made on medicines applications but the full fee is then payable. Some small and medium sized companies can apply for full payment waivers.