PARLIAMENTARY INQUIRY QUESTION ON NOTICE

Department of Health

Standing Committee on Health, Aged Care and Sport

Inquiry into approval process for new drugs and medical technologies in Australia

Written Question on Notice, 23 June 2021

PDR Number: IQ21-000108

Planning future operations that do not rely solely on cost recovery

Written

Member: Trent Zimmerman

Question:

General
1. The Committee has received a lot of evidence about the new drugs and medical technologies that are available overseas and on the horizon.
- Do you think it would be beneficial to consider a new way for the TGA to operate into the future that didn't solely rely on cost recovery?

Answer:

While the TGA's activities are primarily cost recovered from industry fees and charges, a small amount of appropriation funding is provided for other activities. For example in the 2019/20 Mid-Year Economic and Financial Outlook statement, the Government provided $33 million over four years (including $6.6 million in 2020/21) for work on improvement of patient safety through regulatory measures for opioids and to partially defray the costs of the TGA Special Access Scheme, Orphan Drugs Program and mandatory reporting of shortages of critical medicines

There are some activities that may not be appropriately cost recovered under Australian Government Cost Recovery Guidelines (www.finance.gov.au/publications/resource-management-guides/australian-government-cost-recovery-guidelines-rmg-304) because they cannot be attributed to individual TGA sponsors, or it would be unreasonable or inefficient to cost recover (e.g. from individual terminally ill patients in the case of SAS A).

Examples that have been identified in recent consultancies undertaken by KPMG and Noetic include:

- Horizon scanning on new medicines and medical technologies
- Provision of early scientific advice for new and emerging technologies
- Regulatory policy development for new and emerging technologies
- Community and healthcare practitioner education and communications
- Authorised Prescriber Scheme for medicines, medical devices and biologicals
- Special Access Scheme for medicines, medical devices and biologicals
- Orphan Drugs Scheme
- Management of Medicines Shortages
- Chemicals scheduling
- Medicines scheduling
- SME Assist (advice scheme for small businesses and research institutes)
- Medical device application audits initiated by TGA to check product safety or performance
- Good Clinical Practice inspections initiated by TGA to check the conduct of clinical trials
- Compliance management of products that are not on the Australian Register of Therapeutic Goods

It would be a decision for government, and not for officials, to determine whether changes to TGA's funding model are appropriate, and if so how these activities should be funded.

PARLIAMENTARY INQUIRY QUESTION ON NOTICE

Department of Health

Standing Committee on Health, Aged Care and Sport

Inquiry into approval process for new drugs and medical technologies in Australia

Written Question on Notice, 23 June 2021

PDR Number: IQ21-000109

Incentives for repurposing medicines for other drugs and rare diseases

Written

Member: Trent Zimmerman

Question:

Repurposing medicines
10. Has the Department considered ways to provide incentives for the repurposing of medicines for any drugs and in particular rare diseases?

Answer:

The Department undertook a public consultation in February-March 2021 to seek input on how to enhance repurposing of medicines, including those for rare diseases. A stakeholder workshop was held in Sydney on 21 May 2021 and this will be followed by targeted workshops on 1 and 2 July 2021 to draw out options around three particular medicines as case studies.

To stimulate discussion, the Department released a consultation paper that forwarded a number of options to encourage repurposing. Proposals for incentives as discussed in a consultation paper prepared by the Department, submissions to the consultation and in stakeholder workshops included:

- structured regulatory support for repurposing applicants, including outreach to comparable overseas regulators who had approved relevant indications
- development of regulatory and reimbursement guidance regarding clinical trial designs and use of literature based submissions for repurposed medicines

- support for evidence reviews to support the proposed indication (use of the repurposed medicine) or conduct of evidence reviews by the department
- reduction in regulatory and reimbursement fees and charges for repurposed medicines
- provision of exclusivity periods for the applicant, new indications for repurposed off-patient medicines
- early PBAC advice that a medicines could be viewed as suitable for potential reimbursement for a repurposed indication, ahead of making a regulatory submission to the TGA
- simultaneous submission for regulatory and reimbursement evaluation.

Following further stakeholder consultations, incentives and other approaches to stimulate medicines repurposing will be discussed with government for policy consideration.

Any decision to provide incentives for the repurposing of drugs would require a decision by government, and potentially changes to the *Therapeutic Goods Act 1989* and/or the *National Health Act 1953* (and/or the associated charging legislation).

PARLIAMENTARY INQUIRY QUESTION ON NOTICE

Department of Health

Standing Committee on Health, Aged Care and Sport

Inquiry into approval process for new drugs and medical technologies in Australia

Written Question on Notice, 23 June 2021

PDR Number: IQ21-000110

Update on review of the National Medicines Policy

Written

Member: Trent Zimmerman

Question:

Can you update the Committee on where the review of the National Medicines Policy is up to?

Answer:

The Review of the National Medicines Policy (NMP) will commence in August 2021.

Professor Michael Kidd AM will Chair the Review Committee. Further information, including the membership of the Review Committee and the Terms of Reference will be announed prior to the Review's commencement. This information will be made available on Health's website at:
www1.health.gov.au/internet/main/publishing.nsf/Content/National+Medicines+Policy-1.

PARLIAMENTARY INQUIRY QUESTION ON NOTICE

Department of Health

Standing Committee on Health, Aged Care and Sport

Inquiry into approval process for new drugs and medical technologies in Australia

Written Question on Notice, 23 June 2021

PDR Number: IQ21-000112

Submissions resubmitted to the PBAC

Written

Member: Trent Zimmerman

Question:

What approximate percentage of submissions need to be resubmitted to the PBAC because they don't get it right the first time?

What incentives could be considered to assist in receiving applications that meet the requirements the first time?

Answer:

Q1. From March 2020 to March 2021 PBAC meetings inclusive, 38 out of 132 (29 per cent) first time submissions received a 'not recommended' outcome from the PBAC. First time submissions are those that are listed on item five (new drug applications) and item six (requests for changes to listings) of the PBAC meeting agenda.

A PBAC decision to not recommend a Pharmaceutical Benefits Scheme (PBS) listing does not necessarily represent the PBAC's final views on the merits of a medicine. Sponsors may resubmit addressing the concerns previously raised by the PBAC. There is not a set maximum number of resubmissions that are permitted or a fixed time period for resubmissions.

Q2. The PBAC makes its recommendation based on the evidence provided in an applicant's submission, taking into account the medical conditions for which the medicine was registered for use in Australia, its clinical effectiveness, and safety and cost-effectiveness ('value for money') compared with other treatments.

The PBAC Guidelines (https://pbac.pbs.gov.au/information/about-the-guidelines.html) provide detailed instructions on what information is required by the PBAC and the Economic Sub-Committee (ESC) to support consideration of a proposed listing, and the most appropriate form of clinical evidence and economic evaluation for specific submissions. This is supported by Procedure Guidance (www.pbs.gov.au/industry/listing/procedure-guidance/files/Procedure-guidance-for-listing-medicines-on-the-Pharmaceutical-Benefits-Scheme-v2.0.pdf) which details the processes, procedures, timelines and documents required to make a submission to the PBAC.

Various mechanisms are in place to ensure applicants are provided with guidance on making a submission and applicants have an opportunity to respond to commentary on their submission throughout the PBAC process, with the aim of producing higher quality submissions. Applicants have an opportunity to:

- Seek an optional pre-submission meeting for additional guidance and support from the Department before lodging a complex submission
- Provide a written pre-subcommitee response (category one and two) and pre-PBAC response (all submissions) to issues raised during the evaluation
- Request a hearing to directly address the PBAC (category one and two)
- Seek a post-PBAC meeting with the Chair and Deputy Chair for a 'not recommended' decision, or to gain additional context on a positive recommendation in order to proceed to a pricing pathway.

The Department and Medicines Australia have been working together since late 2017 to deliver on commitments under clause 10 of the Strategic Agreement (www.health.gov.au/internet/main/publishing.nsf/Content/landmark-compact-Medicines-Aust) to improve the transparency, efficiency and timeliness of PBS listing processes. Stage one PBS process improvements introduced changes to pre-submission meetings, an intent to apply step for submissions and transparent pricing pathways. This led to a reduction in the time to listing following a positive recommendation by an average of 3.4 months[1] in 2019-20.

Stage two PBS process improvements commenced on 1 January 2021 with the overall objective of reducing the number of resubmissions by 50 per cent through changes to initial submission categories and the introduction of new resubmission pathways. In addition, the Government committed a total $36 million as part of the 2021-22 Budget to expand the Department's Health Products Portal functionality to streamline PBS application processes for applicants.

[1] *2015-17 baseline of 213 days (7.1months) minus the average days to listing of 112.5 days = 100.5 days divided by 30 days = 3.35 months, rounded 3.4 months*

PARLIAMENTARY INQUIRY QUESTION ON NOTICE

Department of Health

Standing Committee on Health, Aged Care and Sport

Inquiry into approval process for new drugs and medical technologies in Australia

Written Question on Notice, 23 June 2021

PDR Number: IQ21-000113

Update on stage 2 of the PBS process improvements that have been implemented since January 2021?

Written

Member: Trent Zimmerman

Question:

PBAC
Have you considered options for having in place a sliding scale for PBAC submission fees depending on the size of the pharmaceutical company?

Answer:

The Department previously has been asked to consider a sliding scale of fees based on company size. As a government agency, the Department must follow the Government's cost recovery policy set out in the Australian Government Charging Framework and the Cost Recovery Guidelines (the CRGs), which specify the framework for design, implementation and review of regulatory charging activities, consistent with the *Public Governance, Performance and Accountability Act 2013.* Cost recovery fees are determined through the Government-approved activity based cost model.

In line with the CRGs, the Pharmaceutical Benefits Scheme (PBS) and National Immunisation Program (NIP) cost recovery arrangements only recover the efficient costs of those services directly requested by sponsors. The fees charged reflect the costs and efforts undertaken by the Department, commensurate with each submission type, regardless of company size.

Cost recovery fees are reviewed and indexed annually to ensure that they reflect contemporary costs. PBS/NIP cost recovery arrangements and fees are outlined in the Cost Recovery Implementation Statement (CRIS) which is updated and published twice annually. The *2021-22 CRIS for Listing of medicines on the PBS and vaccines on the NIP* is available on the PBS cost recovery website at: www.pbs.gov.au/info/industry/listing/elements/fees-and-charges.

Part five of the *National Health (Pharmaceuticals and Vaccines—Cost Recovery) Regulations 2009* (the Regulations) provide a mechanism for companies to apply for a fee waiver or a fee exemption for all PBS and NIP fees relating to PBAC submission services and ATAGI pre-submission advice, with the exception of fees for optional pre-submission meetings.

Fee exemptions apply to all applications that meet the criteria set out in the Regulations. Fee waivers are granted at the discretion of the Secretary or a delegate where an applicant demonstrates that their application is in the public interest and that cost recovery fees would genuinely make the application financially unviable.

Further information on how to apply for a fee waiver or fee exemption can be found on the PBS cost recovery website at: www.pbs.gov.au/info/industry/listing/elements/fees-and-charges and in Section eight of the *PBS/NIP Cost Recovery Administrative Guidelines: Information for Applicants* which is also available on the website.

PARLIAMENTARY INQUIRY QUESTION ON NOTICE

Department of Health

Standing Committee on Health, Aged Care and Sport

Inquiry into approval process for new drugs and medical technologies in Australia

Written Question on Notice, 23 June 2021

PDR Number: IQ21-000114

Streamlined re-application process for existing PBS listing

Written

Member: Trent Zimmerman

Question:

Q1. Has the Department considered streamlining the re-application process for companies wishing to extend or slightly change an existing successful PBS listing?

Q2. Has the Department considered varying the fee structure to accommodate an already successful listing that may benefit from updating?

Answer:

Q1. Yes. The second stage of Pharmaceutical Benefits Scheme (PBS) process improvements (Stage two changes) developed with industry under the current medicines Strategic Agreements commenced in January 2021.

Stage two changes included the introduction of revised submission categories for initial submissions including those seeking an extension or change to an existing PBS listing. Four new resubmission pathways were also introduced in January 2021. These new categories and resubmission pathways were developed in consultation with industry to streamline PBS listing processes.

A request to change or vary an existing PBS listing, or a request to vary a positive recommendation by the Pharmaceutical Benefits Advisory Committee (PBAC), is made through the relevant submission category set out in the Procedure Guidance published at: www.pbs.gov.au/info/industry/listing/listing-steps and outlined in the table below.

Initial submission categories	
Category 1	A request for PBS or National Immunisation Program (NIP) listing of one or more of the following: • A first in class medicine or vaccine, and/or a medicine or vaccine for a new population. OR • A drug with a co-dependent technology that requires an integrated co-dependent submission to the PBAC and MSAC. OR • A drug or designated vaccine with a TGA Provisional determination related to the proposed population.
Category 2	A request for PBS or NIP listing of a new medicine or new vaccine, a new indication of a currently listed medicine or vaccine, or to make material changes to a currently listed indication that does not meet the criteria for a Category 1 submission.
Category 3	A request to change existing listings that do not change the population or cost-effectiveness of the medicine or vaccine that do not meet the criteria for a Category 4 submission.
Category 4	A request for one or more of the following: • Listing of a new pharmaceutical item of a listed medicine. • Consideration as an exempt item (as per subsection 84AH of the *National Health Act 1953*). • Including a listed medicine on the prescriber bag, or varying an existing prescriber bag listing. • A change/new manner of administration of a listed medicine. • A change to the maximum quantity and/or number of repeats of a listed medicine. • A change or addition to the prescriber type(s) of a listed medicine.

Q2. Yes. Following Government approval through the 2020-21 Budget, revised cost recovery arrangements came into effect on 1 January 2021 to support the second stage of PBS process improvements. Evaluation fees vary depending on the submission category nominated by the applicant. The fee for each submission category reflects the work effort of the Department and the PBAC based on the complexity of the different categories of submissions.

The current Departmental fee structure is based on activity based cost model approved by the Department of Finance and the Government. This aligns with the Australian Government Charging Framework and the Cost Recovery Guidelines (the CRGs) which set out the framework under which Government entities design, implement and review regulatory charging activities, consistent with the *Public Governance, Performance and Accountability Act 2013.*

PARLIAMENTARY INQUIRY QUESTION ON NOTICE

Department of Health

Standing Committee on Health, Aged Care and Sport

Inquiry into approval process for new drugs and medical technologies in Australia

Written Question on Notice, 23 June 2021

PDR Number: IQ21-000115

Changes to the MSAC

Written

Member: Trent Zimmerman

Question:

During the inquiry many witnesses called for MSAC to be reformed and have its own legislation in order to define its role, decision making process, stakeholder input and accountability.

- Do you agree that MSAC requires some immediate changes and what is currently being done by the Department in this space?

Answer:

The Medical Services Advisory Committee (MSAC) is a non-statutory committee, established by the Government in 1998. This provides MSAC with a level of flexibility which allows it to be allocated new functions and areas of responsibility by the Government, which can help to respond to emerging technologies and health technology assessment (HTA) needs.

For example, requirements under the National Health Reform Agreement Addendum 2020-25 for MSAC to conduct assessments of Highly Specialised Therapies delivered in public hospitals on behalf of Commonwealth, state, and territory governments were able to be supported with a minor change to MSAC's terms of reference.

Similarly, MSAC's flexible assessment pathways allow factors such as application complexity, applicant capacity, and the variable nature of the HTA assessment that is required for different medical and surgical techniques, products and therapies and diagnostic techniques and products, to be taken into account in determining the most appropriate assessment pathway.

New MSAC Guidelines recently published at: www.msac.gov.au/internet/msac/publishing.nsf/Content/MSAC-Guidelines, which better align MSAC assessment methods with best practice in HTA for therapeutic and investigative technologies, taking account of input from stakeholders.

The new Guidelines are forward thinking and applicable to the range of technologies and services MSAC will likely consider into the future. The updated MSAC Guidelines provide guidance for newer technologies, including genetic testing for heritable diseases and other screening tests.

These aim to provide applicants with clarity and certainty about the assessment methods, which in turn will mean simpler and more successful applications. The Government is committed to continuing to improve MSAC processes, including in respect of stakeholder input, communication and transparency.

From 1 July 2021, revised MSAC consultation processes took effect to improve stakeholder input, provide procedural fairness and improve transparency. A new tab on www.msac.gov.au 'engaging with MSAC' was added and includes improved information for applicants at: www.msac.gov.au/internet/msac/publishing.nsf/Content/Information-for-Applicants on engaging with MSAC and enhanced information on the MSAC consultation process at: www.msac.gov.au/internet/msac/publishing.nsf/Content/MSAC-Consultation-Process, including frequently asked questions at: www.msac.gov.au/internet/msac/publishing.nsf/Content/frequently-asked-questions#MSAC-consultation-FAQ. This is in addition to progressive improvements in engagement and mentoring opportunities for patients and their respective organisations supported by the Department's Consumer Evidence and Engagement Unit.

The expansion of the Health Products Portal, currently used for applications for PBS listing, will provide a single, easy to use place where applicants can apply and track their applications to MSAC and is an opportunity for further process improvements.

In addition, the Department is developing options for improvements to MSAC processes and the potential introduction of cost recovery arrangements to address stakeholder feedback on the need for improved clarity, transparency, and certainty of timeframes.

PARLIAMENTARY INQUIRY QUESTION ON NOTICE

Department of Health

Standing Committee on Health, Aged Care and Sport

Inquiry into approval process for new drugs and medical technologies in Australia

Written Question on Notice, 23 June 2021

PDR Number: IQ21-000116

Assessment of Medicines on the LSDP

Written

Member: Trent Zimmerman

Question:

LSDP

Can you explain the pathways required for a medicine to get assessed on the LSDP?

- Why must all medicines apply to the PBAC first?

- What are the reasons for not having a stand alone pathway for LSDP?

- Have you considered streamlining this process?

Answer:

The Pharmaceutical Benefits Scheme (PBS) is the long-standing national reimbursement program for medicines, consisting of over 5,300 branded products. Maintaining a systematic approach to assessment against the requirements of this scheme is important in order to avoid fragmentation and firstly consider whether new treatments can be made available under the PBS.

This process uses the well-established expertise of the Pharmaceutical Benefits Advisory Committee (PBAC) in assessing comparative safety, clinical effectiveness and cost, avoiding the need to replicate this level of specialised expertise in multiple committees supporting multiple programs. PBAC advice about comparative safety, effectiveness and cost is available for consideration where sponsors of medicines go on to apply for LSDP listing. The PBAC advice forms the basis of the initial LSDP program consideration.

The PBAC has legislated functions under its enabling legislation (the *National Health Act 1953*) and cannot perform other functions at the request of the Government, such as providing advice on other funding programs like the Life Saving Drugs Program (LSDP).

In 2014, the Minister for Health announced a review of the LSDP with the objective of reviewing the access, equity, value for money and future administration of the program.

In response to this review, the Government decided to retain and improve the LSDP as a standalone program, rather than transitioning it to a special program under section 100 of the *National Health Act 1953*, within the legislated functions of the PBAC.

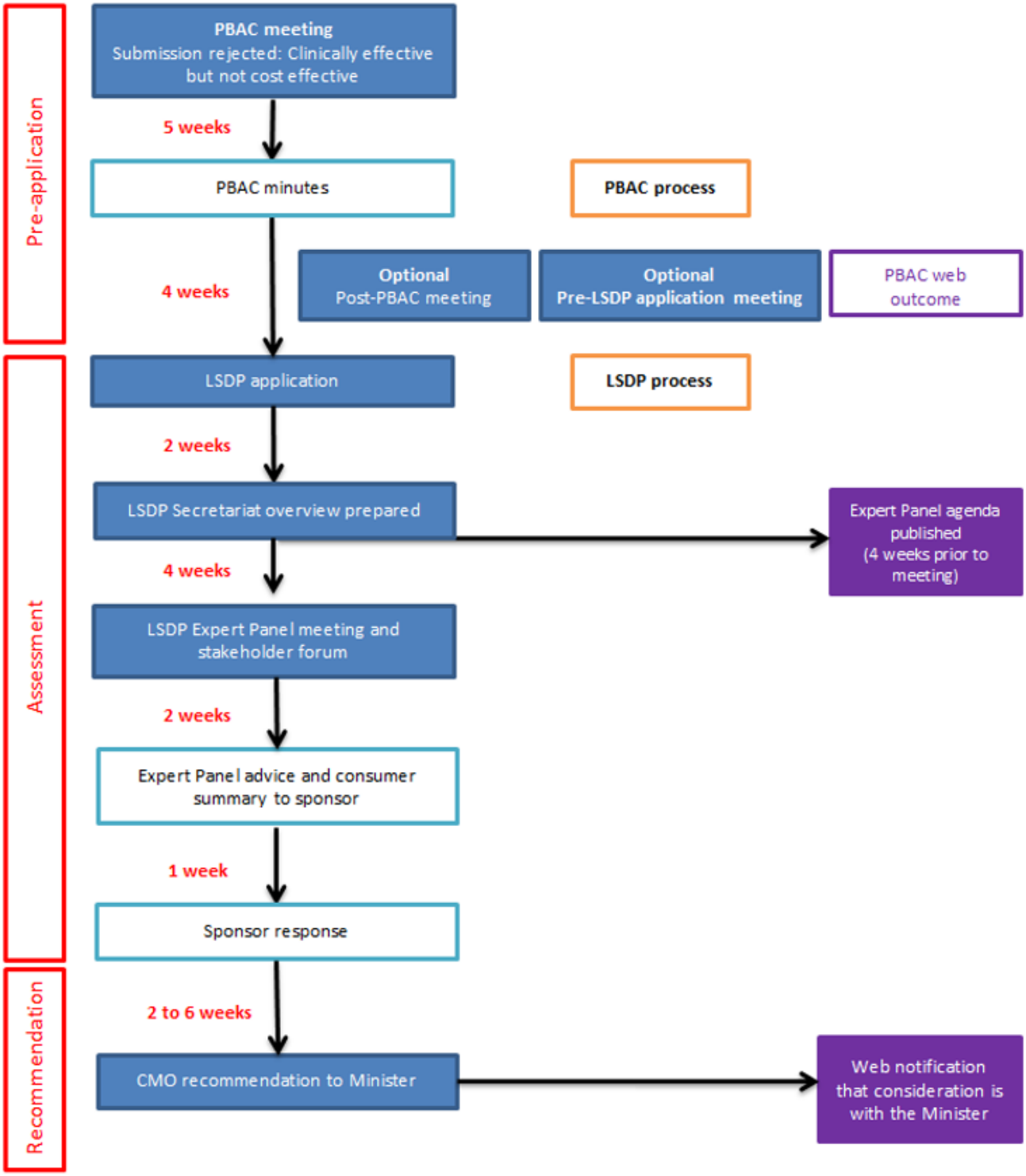
As a result of the review, the Government and industry, represented by Medicines Australia, entered into an agreement in May 2018 to provide policy stability, transparency and certainty for the rare diseases medicines sector. The *Procedure guidance for medicines funded through the Life Saving Drugs Program (LSDP)* outlines the processes for consideration and listing of new medicines for subsidy through the LSDP and is summarised at **Attachment A**.

Changes to the LSDP introduced through this agreement included measures to streamline its administration, the establishment of the LSDP Expert Panel and introduction of 24 month review of usage and financial costs for all medications listed on the program to ensure the program remains sustainable into the future. Changes to LSDP arrangements and listings are considered by Government, with advice and recommendations from the Chief Medical Officer.

The recommendations of a review of the LSDP medicines, completed by the LSDP Expert Panel in late 2020, are currently being considered by Government. Information on the work of the Expert Panel is available on the Department website: www.health.gov.au/committees-and-groups/life-saving-drugs-program-expert-panel#medicine-reviews

Attachment A

PROCEDURE FOR CONSIDERATION OF NEW MEDICINES FOR SUBSIDY THROUGH THE LSDP



PARLIAMENTARY INQUIRY QUESTION ON NOTICE

Department of Health

Standing Committee on Health, Aged Care and Sport

Inquiry into approval process for new drugs and medical technologies in Australia

Written Question on Notice, 23 June 2021

PDR Number: IQ21-000117

Growth of LSDP

Written

Member: Trent Zimmerman

Question:

What sort of growth have you seen for LSDP over the past 5-10 years?

Answer:

The Australian Government funds life-saving medicines for very rare and life-threatening medical conditions through the Life Saving Drugs Program (LSDP). There are currently sixteen medicines available to eligible patients for the treatment of ten conditions (see **Attachment A** for information about growth in the number of funded treatments over time).

Expenditure on the program from 2010 to 2021 is provided in the table below:

Financial Year	Total Life Saving Drug Program Expenditure (GST exclusive, $m)
2010/11	$46.9
2011/12	$70.7
2012/13	$78.6
2013/14	$77.3
2014/15	$85.9
2015/16	$102.2
2016/17	$115.7
2017/18	$126.4
2018/19	$133.7
2019/20	$129.2
2020/21	$137.2

In 2010-11, 210 patients were supported to receive medicines funded by the LSDP.
At 30 June 2016, there were a total of 335 patients accessing medicines via the LSDP and this figure has grown to a total of 463 patients at 30 June 2021.

Generally, once a patient commences treatment on the program they remain on the program for the remainder of their life unless they discontinue treatment for clinical reasons or become part of a clinical trial process.

Attachment A

Condition	Medicine	Sponsor	Financial Year of inclusion
A Gaucher disease	Cerezyme® (imiglucerase)	Sanofi	1999-00
	Zavesca® (miglustat)	Actelion	2009-10
	VPRIV® (velaglucerase)	Shire/Takeda	2012-13
	Elelyso® (taliglucerase)	Pfizer	2015-16
Fabry disease	Replagal® (agalsidase alfa)	Shire/Takeda	2004-05
	Fabrazyme® (agalsidase beta)	Sanofi	2004-05
	Galafold® (migalastat)	Amicus	2018-19
Mucopolysaccharidosis Type I	Aldurazyme® (laronidase)	Sanofi	2007-08
Mucopolysaccharidosis Type II	Elaprase® (idursulfase)	Sanofi	2008-09
Mucopolysaccharidosis Type VI	Naglazyme® (galsulfase)	BioMarin	2008-09
Mucopolysaccharidosis Type IVA	Vimizim® (elosulfase alfa)	BioMarin	2017-18
Infantile-onset Pompe disease	Myozyme® (alglucosidase alfa)	Sanofi	2009-10
Juvenile Late-onset Pompe disease	Myozyme® (alglucosidase alfa)	Sanofi	2014-15
Adult Late-onset Pompe disease	Myozyme® (alglucosidase alfa)	Sanofi	2015-16
Paroxysmal Nocturnal Haemoglobinuria	Soliris® (eculizumab)	Alexion	2010-11
Hereditary Tyrosinaemia Type 1	Orfadin® (nitisinone)	A.Menarini	2015-16
	Nityr® (generic nitisinone)	Orpharma	2018-19
Batten disease	Brineura® (cerliponase alfa)	BioMarin	2018-19

PARLIAMENTARY INQUIRY QUESTION ON NOTICE

Department of Health

Standing Committee on Health, Aged Care and Sport

Inquiry into approval process for new drugs and medical technologies in Australia

Written Question on Notice, 23 June 2021

PDR Number: IQ21-000118

Availability and cost of some medical technologies in public hospital not always in private hospitals

Written

Member: Trent Zimmerman

Question:

There was some concern raised by witnesses about the availability and cost of some medical technologies that were available in public hospitals and the same medical devices weren't always available in the private hospitals and came at a cost for private patients.

- Can you elaborate on this issue and tell the Committee what the Department is currently doing in this space to ensure greater access and equity to medical devices and technologies for all Australians?

Answer:

The Prostheses List is a schedule to the *Private Health Insurance (Prostheses) Rules*. It sets out the minimum benefits a private health insurer must pay for listed prostheses when provided to a privately insured patient (who has appropriate insurance coverage) as part of an episode of hospital (or hospital substitute) treatment in certain prescribed circumstances. As such, the Prostheses List is a reimbursement mechanism, it does not guarantee issues of supply or availability. A product that is not listed on the Prostheses List may still be used for private patients but there will be no obligation for private health insurers to cover the cost of the device and a cost may be incurred by the patient if it is used in a private setting.

In order for a product to be listed on the Prostheses List, a medical device company (known as a 'sponsor') makes an application to the Minister for Health, through the Department, to list its product. The product undergoes an assessment process, considering its clinical and cost-effectiveness, and the Minister (or delegate) makes a decision based on the assessment recommendations about whether or not the product should be listed. If the Minister makes a determination that the product should be listed on the Prostheses List, it will be included in the next remake of the Rules. The Rules are generally made three times a year. Because of the assessment process, some products may be available in the public system ahead of when it is eligible for reimbursement through the Prostheses List arrangements. Other products may not satisfy the clinical and cost effectiveness listing requirements.

As part of the suite of reforms as announced in the 2021-22 Budget Measure, *Modernising and Improving the Private Health Insurance Prostheses List*, it is intended that the purpose and scope of the Prostheses List will be clarified. This may see some technologies become eligible for listing on the Prostheses List that are not currently eligible and, in particular, specific purpose, non-implanted devices. Currently only implanted devices are eligible for listing.

PARLIAMENTARY INQUIRY QUESTION ON NOTICE

Department of Health

Standing Committee on Health, Aged Care and Sport

Inquiry into approval process for new drugs and medical technologies in Australia

Written Question on Notice, 23 June 2021

PDR Number: IQ21-000119

Difficulties for combination therapies that require applications to both PBAC and MSAC

Written

Member: Trent Zimmerman

Question:

Combination therapies

The Committee has heard about difficulties that occur for combination therapies that may have a medicine and a technology that requires an application for both the PBAC and MSAC pathways.

How can your HTA processes be streamlined for combination therapies in the future?

Answer:

Health technologies are codependent if their use needs to be combined (either sequentially or simultaneously) to achieve or improve the intended clinical effect of either technology. Combinations of medicines for Pharmaceutical Benefits Scheme listing alone can be considered by the Pharmaceutical Benefits Advisory Committee (PBAC), and codependent health technologies suitable for Medicare listing alone can be considered by the Medical Services Advisory Committee (MSAC). In some cases, codependent health technologies seek funding through both the Pharmaceutical Benefits Scheme (PBS) and Medicare, and these need to be considered by PBAC and MSAC in a coordinated manner.

A process for the management of codependent applications through PBAC and MSAC has been in place since the 2010 Health Technology Assessment Review. Since then, enhancements to the HTA process for codependent technologies have focused on supporting an integrated approach to reduce duplication of effort across the respective committees, and to minimise potential barriers for patients to have to pay for one health technology when the other is subsidised.

The integrated codependent submission process allows for the preparation of a single evaluation document for use by both MSAC and PBAC. This evaluation document is considered at a joint meeting of the Economics Subcommittee (ESC) of PBAC and the ESC of MSAC, and a single Joint ESCs Advice document is prepared for PBAC and MSAC. PBAC meets three weeks before MSAC, which gives enough time for PBAC to raise any questions if needed for MSAC consideration, for the applicant to comment on the questions and for MSAC to consider its advice.

It has been suggested that this three week period could be avoided by combining PBAC and MSAC. This would require legislative change, and could disadvantage applications to these committees which are not codependent. Integrated codependent submissions comprise a minority of the workload for both committees.

The integrated codependent submission process was successfully used to support MSAC and PBAC's recent consideration of *BRCA* genetic testing to determine eligibility for olaparib maintenance therapy in patients with platinum-sensitive relapsed ovarian cancer.

In some circumstances, a streamlined codependent submission may replace the fully integrated codependent submission. This is where one of the committees considers the health technology relevant to it, knowing that the other committee has already signalled its intention to support the application for the codependent health technology. If the first committee supports its health technology, an expedited process is used to enable the other committee to formalise its support for the other health technology.

PARLIAMENTARY INQUIRY QUESTION ON NOTICE

Department of Health

Standing Committee on Health, Aged Care and Sport

Inquiry into approval process for new drugs and medical technologies in Australia

Written Question on Notice, 23 June 2021

PDR Number: IQ21-000120

Inclusion of patient voice into HTA pathways

Written

Member: Trent Zimmerman

Question:

Patient voice

The Committee received a lot of evidence from Patients and Patient Advocacy groups calling for the establishment of a process to incorporate the patient voice into the PBAC and MSAC pathways.
- Please discuss what the Department currently has available to incorporate the patient voice into HTA pathways and what if any changes are under consideration to incorporate more patient voice, especially for the PBAC, MSAC and LSDP

Answer:

As health technology assessment (HTA) procedures for HTA committees have developed over time, processes to elicit patient input and evidence, to inform committee discussions and considerations, have also developed.

Opportunities for patients and their respective groups or organisations to engage and participate in HTA assessment processes, including those supporting the Pharmaceutical Benefits Advisory Committee (PBAC) and Medical Services Advisory Committee (MSAC) pathways include:

- Direct input through consumer comments made to the committees.
- Invitations to present in person at specific hearings.

- Representation in expert clinical consultations about specific submission items.
- Representation and input to formal stakeholder meetings and public consultations.

In 2020, the PBAC alone was contacted by over 2,200 individuals providing comments on various agenda items across the three main meetings of the committee of that year. From 1 July 2021, revised MSAC consultation processes took effect to improve opportunities for stakeholder input, provide procedural fairness and improve transparency.

The Government has resourced development work to better support consumer representation, and to offer more opportunities for patient involvement in providing consumer evidence and input to HTA committee deliberations.

Since 2019 the Consumer Evidence and Engagement Unit (CEEU) has been established within the Department of Health, to support broader consumer participation strategies. This has included facilitating in-depth interviews and enhanced consultation with patient groups.

The CEEU has developed mentoring programs to enhance the capacity and knowledge of consumer representatives on the various HTA committees and their sub-committees. The CEEU is also considering the consumer engagement approaches of the United Kingdom's National Institute of Health and Care Excellence (NICE) and other HTA bodies, in terms of application to the Australian health system and several projects have begun exploring adoption of similar approaches.

In 2021 the Unit will be evaluating the first pilot of a mentoring project for HTA committee consumer members, initiated to address the need for consumer representatives on HTA committees to be better supported in consumer engagement. Recommendations from this evaluation will guide the next iteration of the mentoring program and enhance the involvement of consumer members in the work of the expert HTA committees.

The CEEU is now exploring ways to enhance the transparency of HTA processes further. A current pilot project underway for PBAC assessment is focussed on sponsor submission summaries being developed in more accessible language by a sponsor and provided to the relevant patient groups, to assist in their understanding of the details of a submission being made to the PBAC. It is expected that this will allow patients and patient groups to provide more considered and informed submissions to the PBAC. This pilot is due for evaluation in the final quarter of 2021.

The Life Saving Drugs Program Expert Panel (the Panel) welcomes input from the public, including patients, carers, family members, treating physicians and advocates as part of its listing and review processes.

Meeting agendas are published four weeks prior to the relevant meeting. Stakeholders can email input to the secretariat two weeks before the meeting. Full details of the consumer submission process for listing and review processes can be found in the Program's procedure guidance: www.health.gov.au/resources/publications/procedure-guidance-for-medicines-funded-through-the-life-saving-drugs-program-lsdp.

The Panel also has an appointed consumer nominee who has significant experience in rare disease consumer advocacy to ensure the patient voice is considered in the context of HTA. More information on the Panel is available on the Department's website: www.health.gov.au/committees-and-groups/life-saving-drugs-program-expert-panel.

PARLIAMENTARY INQUIRY QUESTION ON NOTICE

Department of Health

Standing Committee on Health, Aged Care and Sport

Inquiry into approval process for new drugs and medical technologies in Australia

Written Question on Notice, 23 June 2021

PDR Number: IQ21-000121

Activities to encourage clinical trials in Australia:

Written

Member: Trent Zimmerman

Question:

Clinical trials

Is there any Commonwealth activity at the moment to actively encourage companies to bring clinical trials to Australia?

Answer:

Australia has the objective of being a preferred destination for clinical trials and the industry represents a significant opportunity for Australia. It is estimated that clinical trials contribute $1.4 billion a year to the economy and the sector has been identified as a potential growth area for Australia.

The recent MTPConnect Australia's Clinical Trials Sector report noted steady growth in the sector. A range of factors have contributed to strong activity and interest, including (but not limited to) continuation of the generous Research and Development (R&D) Tax Incentive and the Clinical Trials Notification (CTN) scheme, and our strong reputation for high quality trials and research.

Australia also has the opportunity to show international leadership and foster innovation due to Australia's response to the COVID-19 pandemic and low levels of transmission. Australia hosted at least seven First in Human COVID-19 vaccine trials in the last 18 months, and some companies also noted increased queries on late phase trials where sponsors were considering shifting trials from Europe and USA due to multiple waves of COVID-19, which places Australia well to work with other countries and industry in the coming years.

The Australian Government is taking a multi-pronged approach to encourage clinical trials in Australia, taking into consideration the current environment and the impacts of the COVID-19 pandemic. This includes: international promotion; funding to attract international clinical trials and research, investigators and investment; and streamlining the operating environment and improving processes to make it easier to undertake trials in Australia.

Activities have been underway to promote Australian clinical trials to global audiences. Support for qualified clients (Contract Development and Manufacturing Organisations (CDMOs), Contract Research Organisations (CROs), sites etc.) in business development activities has continued via tailored and targeted "Excelerate" services, aimed at attracting more inbound industry sponsored trials into Australia from major sources such as North America, Europe, Greater China, Singapore and Korea. Virtual roadshows promoting Australia as an ideal destination for clinical trials to potential sponsors in Greater China, Korea and the USA were delivered in partnership with industry stakeholders, with 300 industry delegates attending the sessions from these regions. Austrade led a strong Team Australia delegation (hybrid) to Bio Korea 2021 with all major states and MTPConnect, and involving both physical pavilion and virtual Australia capability promotion activities. Clinical trials was one of the key areas of focus during a dedicated "Australia Korea Open Innovation" forum. Thirty five Australian businesses participated in Bio Korea this year.

The Global Business and Talent Attraction Taskforce is working to attract high yield businesses and highly talented individuals to Australia. Health industries and medical technology is one of the priority sectors being targeted, this includes marquee enterprises involved in the design and management of clinical trials.

The Australian Government is also providing direct investment in the clinical trials sector to encourage companies to undertake clinical trials in Australia. This includes through the Biomedical Translation Fund (BTF) which invests in promising biomedical discoveries with the aim to address various costs constraints, which may include support for clinical trials in Australia. The Modern Manufacturing Initiative (MMI) Translation grant stream for medical products in part aims to help overcome barriers to commercialisation costs including costs associated with clinical trials. In addition, under the Medical Research Future Fund (MRFF) 10-year plan, $614.2 million (GST exclusive) has been committed to the Clinical Trials Activity initiative, which includes the International Clinical Trial Collaboration (ICTC) grant opportunity. The objectives of this grant opportunity are:

- to utilise clinical trials to promote Australian involvement in international collaborative investigator-initiated clinical trials research through the establishment and co-ordination of clinical trial site/s in Australia
- provide high-quality evidence of the effectiveness of novel health treatments, drugs, or devices in 'usual care' settings, which will support a decision on whether to deliver the intervention in an Australian setting.

There are a number of other initiatives/activities underway or planned in collaboration with jurisdictions to strengthen, streamline and harmonise the operating environment, while also maintaining our reputation for high quality research and improving health outcomes, and maximising our global positioning to support jobs and growth in an innovation economy.

The Australian Government has announced a $6 million (GST exclusive) investment over four years to continue the successful Encouraging More Clinical Trials in Australia initiative, supporting ongoing collaboration with jurisdictions to grow the number of clinical trials run in Australia, while removing red tape from the process.

A priority is to continue to build on recent work to develop and pilot the National Clinical Trials Governance Framework, currently being finalised and widely recognised as a significant and positive reform for the sector. Implementation, anticipated from 2022, will streamline trial approval processes, improve time to trial start-up, improve workforce capacity, reduce administered efficiencies and better engage sponsors. The outcome will be the integration of clinical trials into health service corporate and clinical governance systems and nationally consistent accreditation of clinical trial services under the National Safety and Quality Health Service Standards.

The Australian Government has also announced its intention to develop and establish a One Stop Shop for Clinical Trials and Human Research Approvals in collaboration with all jurisdictions, and a related National Clinical Trials Front Door. The announcement to establish the one stop shop has been applauded by the sector and presents a significant opportunity to achieve a national, interconnected, rapid and streamlined approvals platform and will make it considerably easier to undertake and participate in research in Australia. It builds on international evidence that nationalised platforms are critical to building a stronger and more competitive research sector, and that jurisdictional collaboration is critical to success in federated systems. The National One Stop Shop will facilitate rapid and streamlined approvals and address long-standing challenges with duplication, delays and fragmentation that are unlikely to be otherwise overcome. It will underpin the new nationally consistent approach to accreditation for trials sites in public and private hospitals, and provide reporting functionality that will serve to maintain Australia's reputation for safety and quality in research, and drive quality improvement and strategic positioning.

Through ongoing and effective collaboration with jurisdictions, the Commonwealth Government considers that a harmonised national approach is achievable through the Governance Framework, to incorporate clinical trials into routine health service provision, and the single national platform for approvals – the One Stop Shop.

Delivery will significantly progress Australia's agenda to position itself as global leading destination for trials and research, and assist all governments to collaborate and respond to areas of need in a rapid, coordinated and strategic manner. This will ensure better health for Australians while increasing investment in the sector, contributing to economic recovery, and attracting more trials to Australia. National consultations on these important initiatives are underway.

All Australian governments collaborated to develop urgent guidance for the clinical trials community on trial conduct within the COVID-19 restricted environment. This helped researchers, sponsors, institutions, and ethics committees adapt quickly at the outset of the pandemic. A National Teletrials Compendium was developed through effective cross-jurisdictional collaboration and funding from the Encouraging More Clinical Trials in Australia measure. The Compendium aligns with the minimum standards of the International Council for Harmonisation (ICH) Guideline for Good Clinical Practice (GCP) and the National Clinical Trials Governance Framework, and will support a consistent national approach. It is expected to contribute to growth in the number of teletrials in Australia, and pave the way more international teletrials and clinical trials in future.

The TGA also responded to other challenges that impacted the conduct of clinical trials during the pandemic, including the possible need for participants to stay away from or restrict attendance at clinical trial sites, due to risk of infection or government advice to self-isolate. Several variations to TGA notification requirements to address these circumstances have been activated to reduce the reporting requirements by organisations and trial sites, encouraging ongoing conduct and attracting trials to Australia. Full details of variations due to COVID-19 can be found at: www.tga.gov.au/clinical-trial-processes