Response

2

The Department may, at its discretion, publish part or all of the information provided in your submission on the Department's website or in related documents. If information from your submission is published, the Department may identify you and/or your organisation as the author of the submission. All personal contact details will be removed prior to publishing.

Yes, I consent to my identified submission being published

3

What is your name?

Natashia Coco

7

Please select the type of individual(s) or organisation(s) you represent. Please select all that apply. - Selected Choice

Patient or consumer (or representative organisation)

8.1

What is the name of your organisation? - My organisation is called: - Text

Haemophilia Foundation Australia

9

Are you making feedback on behalf or your organisation?

Your organisation

13

Please select which chapter/s you would like to provide feedback on. You may provide feedback on as many or few chapters as you wish.

1. Transparency, communication, and stakeholder involvement in HTA,2. Health technology funding and assessment pathways,3. Methods for HTA for Australian government subsidy (technical methods),4. Health technology funding and purchasing approaches and managing uncertainty,5. Futureproofing Australia's systems and processes

14

Please select the topics within the chapter(s) you would like to provide feedback on. 1. Transparency, communication and stakeholder involvement in HTA

1.1. Transparency and communication of HTA pathways, processes and decisions,1.2. Consumer, clinician and other stakeholder engagement and consideration in HTA,1.4. State and territory government collaboration in HTA

15

Please select the topics within the chapter(s) you would like to provide feedback on. 2. Health technology funding and assessment pathways

2.1. Streamlining and aligning HTA pathways and advisory committees

16

Please select the topics within the chapter(s) you would like to provide feedback on. 3. Methods for HTA for Australian government subsidy (technical methods)

3.2. Clinical Evaluation Methods,3.3. Economic evaluation

17

Please select the topics within the chapter(s) you would like to provide feedback on. 4. Health Technology funding and purchasing mechanisms and decisions

4.1. Approaches to funding or purchasing new health technologies

18

Please select the topics within the chapter(s) you would like to provide feedback on. 5. Futureproofing our systems and processes

5.2. Establishment of horizon scanning programs to address specific informational needs within HTA and the health system

21

Taking all Options within this section: 1.1. Transparency, communication and stakeholder involvement in HTA into account.

Overall, to what extent could the options (if implemented) address the issues that relate to them?

Mostly address the issue(s)

22

If you would like to expand on your answer above you can do so below:

Haemophilia Foundation Australia (HFA) represents more than 7,400 Australians diagnosed with bleeding disorders, including haemophilia, von Willebrand disease (VWD), rare clotting factor deficiencies, inherited platelet disorders and other rare bleeding disorders. We welcome the opportunity to comment on the Australian Government Health Technology Assessment Policy and Methods Review.

We are in a time of great innovation in therapies for bleeding disorders, with the potential to deliver life-changing improvements to health outcomes and quality of life. Ensuring that Australians with bleeding disorders can access new health technologies as soon as possible and in an equitable way will be crucial to their healthcare into the future. At the same time, as always, the Australian regulatory system needs to take into account the safety and effectiveness of these new technologies, along with a careful assessment of cost-effectiveness and use of taxpayer funding.

We have commented in our previous HTA Review submissions on the importance of an HTA system that is clear, transparent and accountable for all stakeholders, including consumers, and commend the Review for developing options to address some of the issues. Our comments on specific options are in the tables below.

The background on issues that we have been concerned about is at the end of this section.

25

If you would like to expand on your answer above you can do so below -Publish plain language summaries

Plain language summaries of HTA submissions that are published at the same time as the Agenda will be valuable for engaging consumers with the questions.

Clear and transparent reports of committee deliberations and a plain language explanation of HTA processes and PBAC guidelines will assist greatly with:

'cconsumer understanding of how and when to contribute to the process
'cand how to work with other stakeholders on responding to a decision that is unfavourable or where more information is required.

It will also be important to develop a plain language HTA submission structure for consumers that aligns with the HTA requirements and enables consumers and consumer organisations to gather and report evidence that will have weight with the assessment committee.

General guidelines in plain language and aimed at consumer organisations will also be critical to enabling them to develop and prepare suitable evidence over time. A survey of patient-reported health outcomes, for example the PROBE (Patient Reported Outcomes Burdens Experiences) Study in haemophilia, can take several years to establish, recruit responses and report in a way that is acceptable in an HTA environment. It can also take months or even years to collect relevant patient stories and experiences.

See also our comments in section 1.2.

26

If you would like to expand on your answer above you can do so below -Improvements to the HTA webpage including development of a dashboard

We are pleased to see the proposal of a visual dashboard to communicate the status of health technologies as they progress through the HTA system, and that this will be available at an aggregate and individual drug level and informed by horizon scanning.

Like some other health conditions, therapies in bleeding disorders are evolving rapidly and the community is often aware that their fellow patients in other countries already have access to new therapies and their experiences of them. Access to new therapies in Australia often lags behind other countries with similar health economies and it will help to address community questions if the HTA progress for that therapy or type of therapy is available publicly and in a simple visual way. It will also assist consumer organisations to begin preparation for their submissions in consultation with other stakeholders or to understand how a submission is progressing.

Some questions:

'cWill expected timeframes for each step be published?
'cHow will consumer organisations be alerted to submissions being open to relevant therapies?

While it will be valuable to have the information on a dashboard, there also needs to be a way that consumers can be advised proactively that a relevant consultation is occurring and in a timely way, so that they have time to contribute effectively.

27

Taking all Options within this section: 1.2. Consumer, clinician and other stakeholder engagement and consideration in HTA into account.

Overall, to what extent could the options (if implemented) address the issues that relate to them?

Don't know

28

If you would like to expand on your answer above you can do so below:

While these options provide a good structure for consumer engagement and consideration in HTA, how effective they will be depends on how they are enacted.

Our specific comments and examples are in the tables below.

We are also concerned by the inconsistent approach in the Review to the value and weight of consumer evidence and that this may ultimately mean that consumer evidence is automatically given a low weighting, whether it is included in Real World Evidence in a methodologically sound and rigorous way or not.

For example, we note that in 4.1 Approaches to funding or purchasing new health technologies, the option refers to '"new therapies that offer no advantage in terms of improved efficacy and safety (i.e, no improved health outcomes)'. This very narrow definition of '"health outcomes' excludes the health outcomes beyond specific clinical endpoints, the '"indirect benefits' that may actually be very direct to the patient, the health system as a whole and government and its services. It will be important to be able to measure this in a way that is usable as evidence for HTA, but if the actual HTA process still discounts this evidence, the value to the consumer will be lost.

An example from bleeding disorders is given in the last table in this section.

31

If you would like to expand on your answer above you can do so below -Develop an engagement framework

CONSUMER INPUT IN CLINICAL TRIALS

We value the inclusion of consumer input in clinical trials. Asking the sponsor to report on patient input or experience data will add weight to it in the clinical trial and HTA process. However, it needs to be recognised that if a consumer organisation also reports on this data, it is not necessarily duplication: a consumer organisation is independent of the sponsor and may wish to use the same data to take a different position or approach.

CONSUMER REPRESENTATION IN HTA COMMITTEES

Consumer participation in HTA committee meetings will be valuable, but it needs to be the right consumer representative. Where therapies are specific to a health condition, it will be important for the consumer to have the expertise and experience to represent that disease-specific area and have access to the rich knowledge base and collections of patient experience that already exist or are in development.

Similarly, the right kind of consumer evidence needs to be included. While it will be important to establish a dedicated consumer evidence base and condition/disease repository, it is vital to have patients with the condition at the table for discussion with the Committee as well. Published evidence-based data is only one part of the story: there is the expertise of the patient in relation to experience and the knowledge of projects and approaches that are in the pipeline. This is increasingly important where therapies and practice are evolving rapidly.

32

If you would like to expand on your answer above you can do so below -Strengthen consumer evidence

SPECIALISED PATIENT EXPERTISE
It will be crucial to draw on specialised patient expertise to strengthen consumer evidence.

Bleeding disorders such as haemophilia are a highly specialised area, both for clinicians and for consumers. Patients have lived with the health condition since birth, live with specific symptoms and health outcomes and have been exposed to particular treatments over their lifetime. Their health-related outcomes may have changed over time and their preferred outcomes are equally dynamic, with the impact of new therapies. They contribute to national and international data and they and the patient organisation, Haemophilia Foundation Australia, participate in national and international forums and studies to understand patient needs and improve the patient experience of their specific health condition.

High quality evidence-based data on patients and treatments is collected into the national Australian Bleeding Disorders Registry (ABDR), the clinical system managed by Haemophilia Treatment Centres across Australia, and patients contribute to this via the MyABDR app. HFA is a national member organisation of the World Federation of Hemophilia (WFH) and participates regularly in global forums and data collection activities such as the WFH Global Survey. HFA also leads Australian participation in international patient research studies, such as the highly-regarded PROBE study in haemophilia (see 1.1), which is validated internationally and for Australia.

40

If you would like to expand on your answer above you can do so below:
We welcome the development and implementation of a nationally cohesive approach to HTA as an opportunity to have consistent principles built into the approach across health technologies that are jointly funded by Commonwealth and state/territory governments.

However, this needs to support and enhance rather than weaken or replace the existing National Blood Arrangements and National Framework to Manage the Treatment and Care of Bleeding Disorders. This is a highly integrated and effective system and is outlined below.

NATIONAL FRAMEWORK

Therapies for bleeding disorders are personalised, complex and require specialised oversight. Most represent a high cost for governments. To ensure well-managed and efficient access to these therapies in Australia we are fortunate to have a well-established national framework to manage the
treatment and care of bleeding disorders and facilitate purchasing on behalf of governments.

'cApproved therapies for bleeding disorders are supplied at no direct cost to the patient under the NATIONAL BLOOD AGREEMENT.
'cUnder this Agreement, products that are approved for funding by all Australian government health ministers are listed on the NATIONAL PRODUCT LIST and funded 63% by the Commonwealth and 37% by the states and territories.
'cThe NATIONAL BLOOD AUTHORITY (NBA) manages and coordinates arrangements for the supply of blood and blood products and services under the National Blood Agreement for all Australian governments.

43

If you would like to expand on your answer above you can do so below -Development of central standardised data sharing system for utilisation and outcome data
The Framework also integrates Haemophilia Treatment Centres (HTCs) and the Australian Bleeding Disorders Registry:

'cDesignated HAEMOPHILIA TREATMENT CENTRES (HTCs), located in major public hospitals in each state/territory, provide comprehensive care, i.e. specialised multidisciplinary care. HTCs enable best practice care and careful and strong oversight of home therapy. HTCs are recognised internationally as providing the best health outcomes and safety for patients with bleeding disorders.
'cThe AUSTRALIAN BLEEDING DISORDERS REGISTRY (ABDR) is a robust database used to document and manage clinical care in HTCs and for patients to record home therapy and bleeding episodes. It supports the National Blood Authority with data for procurement and supply and provides aggregated research data to evaluate the impact of therapies.

44

If you would like to expand on your answer above you can do so below -Increase opportunities for consultation and work sharing
A great strength of the National Framework is that it fosters collaboration, partnerships, sharing of knowledge and expertise very efficiently and effectively.
'cThere is a long history of STAKEHOLDER PARTNERSHIP AND COLLABORATION between patients, clinicians, governments and industry stakeholders, with recognition that each contribute to best practice health outcomes and cost management.
'cKNOWLEDGE DEVELOPMENT AND PRACTICE IMPROVEMENT: clinical treatment for bleeding disorders is a specialised area of haematology. The specialists who provide treatment and care in HTCs develop and use best practice evidence-based clinical guidelines consistent with international guidelines and participate in international research. This involves both haematologists and other disciplines in the multidisciplinary HTC team, such as nursing, physiotherapy, psychosocial care and laboratory science. Likewise the patient organisation, Haemophilia Foundation Australia (HFA), contributes to this from a consumer perspective.

45

If you would like to expand on your answer above you can do so below -Health technologies that are jointly funded by the Commonwealth and state and territory governments (such as high cost, Highly Specialised Therapies (HSTs) delivered to public hospital inpatients)
COMMITMENT TO INNOVATION AND BEST PRACTICE CARE

A nationally cohesive approach to HTA would rely on a fundamental acknowledgement of the need to support innovation and access to best practice care in the implementation of new therapies.

The National Blood Agreement underpins the current framework and governs blood policy and access to blood products and blood-related products and services in Australia. The current primary objectives of the National Blood Agreement are:

a) To provide an adequate, safe, secure and affordable supply of blood products, blood related products and blood related services in Australia; and
b) To promote safe, high quality management and use of blood products, blood related products and blood related services in Australia.

These objectives are crucial. However, we believe the Agreement objectives should be expanded to include:
'cA commitment to innovation and best-practice care; and
'cRecognition that innovation in therapies can contribute to cost-effectiveness.

We will comment on HORIZON SCANNING in 5.2.

46

Taking all Options within this section: 2.1. Streamlining and aligning HTA pathways and advisory committees into account.

Overall, to what extent could the options (if implemented) address the issues that relate to them?
Don't know

47

If you would like to expand on your answer above you can do so below:
The proposal to '"develop a unified, national, HTA pathway' would be valuable for streamlining some processes but we are unsure how this is intended to work with blood products and the National Blood Agreement.

Crucial to the process would be:

'cDrawing on the expertise of specialist haematologists who have a current caseload of patients with bleeding disorders and can contribute from their expertise and knowledge, clinical networks and their clinical experience and consult with their colleagues, as appropriate.
'cEnsuring the voice of patients with bleeding disorders is also represented at the table to contribute from the patient perspective and knowledge base, including preferred patient outcomes and patient experience.
'cThe submission is then referred to the National Blood Agreement pathway for funding.

Having the right expert clinicians and patients at the table with the Committee will also streamline the process. Hurdles can be managed at the time with their expertise by providing or sourcing relevant advice or data, rather than the matter under question being referred to another round of evaluation.

61

If you would like to expand on your answer above you can do so below -Expanding role of PBAC
Under current National Blood Arrangements the process to consider new bleeding disorders therapies for funding involves several steps.

Streamlining some of these arrangements through a triaging stage and a parallel process for TGA and HTA evaluation would improve the process. However, it will be imperative to accommodate processes such as tendering and not to lose the very valuable elements of the National Blood Arrangements that already exist and have shown themselves to be strategic and cost-effective over the last 25 years.

62

If you would like to expand on your answer above you can do so below -Unified HTA pathway for all health technologies with Commonwealth funding
THE VALUE OF THE NATIONAL BLOOD ARRANGEMENTS

The blood sector is made up of multiple components. Our view is that it requires a coordinated or federated approach to achieve the best outcomes for government payers and patients. With the shared interests and responsibilities across all jurisdictions and nationally, the National Blood Arrangements foster a coordinated and integrated approach to access to treatment and best-practice care, along with a focus on efficiency and cost-effectiveness.

We fully support the role of the National Blood Authority (NBA) as the appropriate body to facilitate the funding process for new therapies for bleeding disorders.

The national framework was built on a decision that blood and defined blood products are not suited to prescribing under the Pharmaceutical Benefits Scheme (PBS). They are specialised therapies that are prescribed under the supervision of Directors of HTCs, who are experienced specialist haematologists, acting jointly with the Australian Haemophilia Centres Directors' Organisation. This takes into account all of the best practice elements of the National Framework and ensures that these highly specialised products are used to treat patients appropriately and safely.

94

Therapies that target biomarkers (e.g. tumour agnostic cancer therapies, therapies that target particular gene alterations)

95

If you would like to expand on your answer above you can do so below -Pharmacogenomic technologies
We note the proposal to develop a statement of principles concerning the access and use of genomic technologies and gene therapies through clinician/patient co-design and that this should include '"people who do not have an immediate vested interest in these technologies'.

We agree that it is important that the process includes a more objective viewpoint. However, in rare diseases these are highly specialised situations and these participants would need careful briefing so that they could understand the patient experiences and the impact on their quality of life and be able to put the value of the new technology in a perspective that is comparative to the existing therapies and patient outcomes.

102

If you would like to expand on your answer above you can do so below -Valuing overall
Similarly to our comment in 3.2, there would need to be a cautious approach to a population representative sample to evaluate value in highly specialised therapies. In particular, we would be concerned about how to provide adequate education in a short timeframe to enable them to understand the therapy and the patient experience in such a highly specialised and sensitive framework.

104

If you would like to expand on your answer above you can do so below:

As discussed in 1.2, we note that this option states '"new therapies that offer no advantage in terms of improved efficacy and safety (i.e, no improved health outcomes) would be required to offer a lower price to be funded' and are concerned at the narrow definition of '"health outcomes' that does not take account of health outcomes and indirect benefits beyond specific clinical endpoints.

How will this approach improve the current situation with new therapies if it can only take into account the very limited morbidity measures and not factor in the value for money of other health outcomes and benefits?

Moreover, if the new therapy is competing in cost with an existing therapy that has been purchased by government at a discounted price, there is an automatic competitive imbalance in the process. Treatment product choice for blood products is limited for Australian patients with bleeding disorders by what is made available on the National Product List under the National Blood Agreement. We support this system and have a high regard for it as it ensures that all Australians with bleeding disorders have access to effective and safe treatments but we are concerned that a purchasing system that is too imbalanced will reduce product choice for Australians, excluding innovative new therapies that may have a great impact on patients' broader health outcomes and quality of life.

141

If you would like to expand on your answer above you can do so below:

Horizon scanning to facilitate timely planning and adoption ahead of TGA sponsor applications is crucial in these times of rapidly evolving therapeutic technologies. It is also important to ensure that there is a disease-specific as well as a broader approach to this in stakeholder input and opportunities for each group to learn from each other.

This option has the potential to build on existing strengths in the blood products area and National Blood Arrangements.

With low patient numbers worldwide, rare diseases are by their nature not conducive to large national clinical studies. International networks in the disease area are often already active in collaborating, sharing data to aggregate larger data results and developing best practice clinical guidelines. They also collaborate to develop and validate health-condition specific benchmarks and evaluation tools.

144

If you would like to expand on your answer above you can do so below -Horizon scanning for advanced therapies (including high cost, HSTs funded through the NHRA) and other potentially disruptive technologies

International horizon scanning to see what therapies are advancing in the pipeline and could have benefits for Australians is standard practice in bleeding disorders. Australia has a history of slow access to innovative therapies, as has been our experience with bleeding disorders. For example, it took nearly three years from registration to funding approval in 2020 for an important innovative therapy for haemophilia A, emicizumab/HemlibraÂ®. This meant that Australian patients with inhibitors and severe haemophilia continued to live in pain, experience bleeding episodes, hospitalisations and poor quality of life while patients in other countries with similar health economies already had access to this therapy for some years.

However, this horizon scanning and monitoring of experience in other similar countries can provide an opportunity for valuable collaborations among stakeholders to prepare the ground for them.

146

If you would like to expand on your answer above you can do so below -Horizon Scanning to help operational and capacity planning for HTA and health systems

Gene therapy in haemophilia is one example where work to prepare the ground to help operational and capacity planning is taking place in Australia, learning from the process in other countries :

'¢Australia has participated in the international clinical trials
'¢The World Federation of Hemophilia (WFH) has developed a Gene Therapy Registry to aggregate clinical results internationally
'¢WFH has also developed a Shared Decision-Making Tool for patients and clinicians considering new haemophilia therapies and this will be tested in Australia
'¢The Australian Haemophilia Centre Directors' Organisation (AHCDO) has developed a model of care to provide equitable best practice access to gene therapy nationally
'¢PROBE international is developing and validating patient reported outcome measures specific to gene therapy in haemophilia, with participation from Australian patients
'¢HFA and Australian clinicians have been looking closely at patient outcomes and experiences reported in Australia and other countries
'¢Australian stakeholders are monitoring the progress of gene therapy for haemophilia through HTA in other countries with similar health economies, consulting with international colleagues on their experience and are considering what may be required for HTA in Australia.

170

Finally, do you have any further comments about the draft Options Paper or consultation you would like to make before submitting your feedback?

The limit on characters in fields made responding appropriately to the questions difficult.

211

Do you have further comments or concerns to add specific to this topic that should be considered? For example, here you can detail any unintended consequences or overlooked considerations if applicable.

Our concerns:

The lack of transparency in the HTA process makes it difficult for a consumer organisation to know how they could best contribute and where the process was challenged because of a lack of evidence or information that they could have provided.

This lack of transparency is compounded by difficulties for a consumer organisation:

'¢Finding out about relevant consultations and their submission deadlines in a timely way so that they can consult and put together an appropriate response.
'¢Information about the HTA process and submissions may be overly technical, making it challenging for a consumer organisation to respond effectively.

Uncertainty about the approval process also causes concern and distress in the community.

'¢Involving the affected patient voice and specialist clinicians in the HTA process at all stages will trigger appropriate contributions from consumers and consumer organisations and assist with stakeholder confidence in the process
'¢More clarity and transparency about the approval process is required, so that the stakeholders, including clinicians, the affected community and industry are clearer about the process and the progress of specific proposals.
-All steps in the evaluation process should be identified and timelines published.
-This includes the timeframes for the public release of recommendations to avoid delays in informing the clinicians and community.
'¢There is a need for clear and accountable timeframes for HTA.

212

Do you have further comments or concerns to add specific to this topic that should be considered? For example, here you can detail any unintended consequences or overlooked considerations if applicable.

The very narrow definition of 'health outcomes' in the Review also discounts very significant broader but related health outcomes.

In new haemophilia therapies we have seen that, as a result of fewer or no bleeds, patient health and strength has improved overall, as they can undertake exercise and gym work without bleeds and can now work or participate in study full-time and without interruptions, leading to a return to work for their parents or partners, improved mental health and much higher quality of life for all generally. This reduces the load on hospitals, with fewer inpatient visits and complications to manage, on mental health services and on government financial assistance; moreover both the patients and their carers are contributing to the national economy as tax payers.

We anticipate that we will be able to measure some of these patient outcomes in the current and future rounds of the PROBE Australia Study and would be concerned if this data was of little value for HTA, even if reported appropriately.

214

Do you have further comments or concerns to add specific to this topic that should be considered? For example, here you can detail any unintended consequences or overlooked considerations if applicable.

221

Do you have further comments or concerns to add specific to this topic that should be considered? For example, here you can detail any unintended consequences or overlooked considerations if applicable.

The NBA brings fragmented blood arrangements together into a national structure so that blood and blood products, including therapies for bleeding disorders, can be managed for all governments in a cost-effective way.

'¢It uses evidence-based tools such as the ABDR for accurate data to evaluate treatment product use and project requirements for supply.
'¢The ABDR also provides a specialised database to evaluate treatment outcomes with real-world data.
'¢Stakeholder collaboration and partnership in providing access to new therapies has ensured the willingness of the patient community to commit to recording home therapy and their treatment product inventory through the MyABDR app. Treatment recording has improved greatly in recent years as a result. This supports the best use and minimises wastage of these high cost treatment products and also enables care that is based on timely and accurate recorded information.

An important aspect of the National Framework is its recognition of the many complementary elements involved in treating people with bleeding disorders. The treatment of bleeding disorders is far more complex than prescribing medication and involves a multidisciplinary team providing comprehensive care. This is demonstrated in the best practice '"Guidelines for the management of haemophilia in Australia' (2016), which is built on the national framework.

252

Do you have further comments or concerns to add specific to this topic that should be considered? For example, here you can detail any unintended consequences or overlooked considerations if applicable.

With its focus on cost-effective procurement from both Australian and international sources and access to high quality data on the affected population and their treatment product needs, the National Blood Authority is well placed to undertake horizon scanning on behalf of government.

The long history of stakeholder partnership and collaboration in the blood sector provides a strong basis for all stakeholders to work together to share knowledge and develop effective strategies to plan for adoption before the HTA process begins.