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. Please publish my submission anonymously (personal and organisational names will be removed and the submission will be marked as '"Name withheld') (Note: if you select this option and your submission contains identifying information, your submission may not be published).
3
What is your name? [redacted]
7
Please select the type of individual(s) or organisation(s) you represent. Please select all that apply. - Selected Choice [redacted]
8.1
What is the name of your organisation? - My organisation is called: - Text [redacted]
9
Are you making feedback on behalf or your organisation? [redacted]
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,1.3. First Nations people involvement 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
18
Please select the topics within the chapter(s) you would like to provide feedback on. 5. Futureproofing our systems and processes 5.4. Mechanisms for continuous review and improvement
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: The WHO recommended goal for iterative co-design with consumers and all CT stakeholders including pharma, an underappreciation that design must be enhanced in terms of meaningful measures and evidence, not only in terms of lived experience - but in mutual discussion of the patient journey including condition features as they are - not as they are published so inaccurately increasingly in the journal literature, including factors such survival rate, which may impact particularly those patients too young to give consent. Are we asking the wrong question, would a better one be about the speed at which we are breaking down this critical consumer partnership barrier despite the scholarship/CT methods of critical consumer partners? Who does it serve to research less well? Surely efficacy is paramount?
23.1
If implemented, overall would these Options have a positive or negative impact on you (/your organisation)? - Publish plain language summaries Positive
23.2
If implemented, overall would these Options have a positive or negative impact on you (/your organisation)? - Improvements to the HTA webpage including development of a dashboard Very positive
25
If you would like to expand on your answer above you can do so below -Publish plain language summaries Publishing plain language summaries: These should be viewed as valuable adjuncts for everyone - those who comprehend and those who consolidate from explaining in succinct clear language or graphically. Jargon may be internalized by consumers with experience but that doesn't equate to eg critical ability to design or appraise research or make new ethical leaps to solve problems of eg. COI declarations for pages Vs our need for independent appraisal.
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 A dashboard would be a welcome central hub or App rather than reinventing the wheel, it should hyperlink from a consistent and colour coded navigational path, to be able to cross reference easily - including regulatory status, international approvals, CT registry and pipeline stages.
33
Taking all Options within this section: 1.3. First Nations people involvement and consideration 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)
35.1
If implemented, overall would these Options have a positive or negative impact on you (/your organisation)? - First Nations peoples partnership in decision making Positive
35.2
If implemented, overall would these Options have a positive or negative impact on you (/your organisation)? - Dedicated resource for HTA submissions and education Positive
37
If you would like to expand on your answer above you can do so below -First Nations peoples partnership in decision making Of course diverse cultures and FN should be represented in the scope of engagement, whether lived experience or critical co-designing partners. To enable access and compliance in engagement in research as expert or participant, decentralized clinical trials, including RWE will need to be normalized/harmonized as a trial arm and technologies and systems customized to adjust for variables.
38
If you would like to expand on your answer above you can do so below -Dedicated resource for HTA submissions and education FN health consumers often have higher risks of chronic diseases and types of eg respiratory infections because of economic or rural and remote circumstances but not necessarily exclusively. A better approach would not be to segregate or target in study applications, which is why I suspect we are not succeeding at closing the health gap, but rather to ensure representation as one group among others, by mandating participant diversity in a clinical trial. For instance treatments targeting fast twitch muscle fibres - the diversity of FN participants will help identify outliers and explain inherited modifiers that impact results for all. More importantly, it might mean that we are not reinforcing fatalistic expectations based on origin stories but objectively identifying correlations which may be circumstantial like location/lifestyle.
39

Taking all Options within this section: 1.4. State and territory government collaboration 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)

41.1

If implemented, overall would these Options have a positive or negative impact on you (/your organisation)? - Development of central standardised data sharing system for utilisation and outcome data

Very positive

41.2

If implemented, overall would these Options have a positive or negative impact on you (/your organisation)? - Increase opportunities for consultation and work sharing

Very positive

41.3

If implemented, overall would these Options have a positive or negative impact on you (/your organisation)? - 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)

Very positive

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

Consider national trends like specialist networks collaborating and establishing networks in addition to genetic registries. For instance...historically neuromuscular registry stakeholders formed a network for associated care and treatment...lately diverse professional/specialty networks are reversing that and seeking registries of their own for evidence in their own fields: cardiac, brain, respiratory/sleep and could focus on archiving images say. These trends will have an impact on the level of interoperability of data sharing for base line or outcomes from post marketing reporting 10yrs hence. If we are changing the course of disease then we can expect very different outcomes ages/stages/progression, we can't rely on the out of date literature to describe conditions/benchmarks accurately...combination therapies and complexity redefines natural history.

44

If you would like to expand on your answer above you can do so below -Increase opportunities for consultation and work sharing

We have national resources like registries but recently a trial site opportunity was missed because companies consulted a jurisdiction/clinic and drew likely the wrong picture of recruitment success - the national tools we develop can't be lost but must be supported and promoted to attract international trial sites and integrated into the central hub..

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?

Mostly address the issue(s)

48.1

If implemented, overall would these Options have a positive or negative impact on you (/your organisation)? - Pathway for drugs for ultra-rare diseases (Life Saving Drugs Program (LSDP))

Neutral

48.2

If implemented, overall would these Options have a positive or negative impact on you (/your organisation)? - Vaccine pathway

Very positive

48.3

If implemented, overall would these Options have a positive or negative impact on you (/your organisation)? - Expanding role of PBAC

Neutral

48.4

If implemented, overall would these Options have a positive or negative impact on you (/your organisation)? - Unified HTA pathway for all health technologies with Commonwealth funding

Very positive

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

This can be better achieved by augmenting these advisory groups with independent critical consumers, independent science reviewers capable of deeper patient journey mapping to factor in genetic, treatment or lifestyle modifiers, uncontrolled bias.

84

Taking all Options within this section: 3.2. Clinical Evaluation Methods into account.

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

Mostly address the issue(s)

86.1

If implemented, overall would these Options have a positive or negative impact on you (/your organisation)? - Overarching principles for adopting methods in Australian HTA

Positive

86.2

If implemented, overall would these Options have a positive or negative impact on you (/your organisation)? - Methods for the assessment of nonrandomised and observational evidence

Positive

86.3

If implemented, overall would these Options have a positive or negative impact on you (/your organisation)? - Methods for the assessment of surrogate endpoints

Positive

86.4

If implemented, overall would these Options have a positive or negative impact on you (/your organisation)? - Generate a curated list of methodologies that are preferred by decision-makers, in collaboration with evaluation groups and sponsors.

Positive

86.5

If implemented, overall would these Options have a positive or negative impact on you (/your organisation)? - Develop an explicit qualitative value framework

Positive

86.6

If implemented, overall would these Options have a positive or negative impact on you (/your organisation)? - Therapies that target biomarkers (e.g. tumour agnostic cancer therapies, therapies that target particular gene alterations)

Positive

86.7

If implemented, overall would these Options have a positive or negative impact on you (/your organisation)? - Pharmacogenomic technologies

Positive

90

If you would like to expand on your answer above you can do so below -Methods for the assessment of nonrandomised and observational evidence

PROMS and RWE using technology are more important - particularly when validated than other methods with inherent bias/confounding or where a study compares apples and oranges. Using score sheets or customized wearables are clear ways to add power to any investigation in the most meaningful sphere of measurable function.

91

If you would like to expand on your answer above you can do so below -Methods for the assessment of surrogate endpoints

Surrogates are required to be validated - why not RWE. It requires greater investment to customize specialized wearables or use expert score cards to exclude non-valid measures but these would add power to any study.

92

If you would like to expand on your answer above you can do so below -Generate a curated list of methodologies that are preferred by decision-makers, in collaboration with evaluation groups and sponsors

Guidance from condition groups can assist in the

152

Taking all Options within this section: 5.4. Mechanisms for continuous review and improvement into account.

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

Mostly address the issue(s)

154.1

If implemented, overall would these Options have a positive or negative impact on you (/your organisation)? - A program of continuous review and improvement for current HTA policies and methods

Very positive

156

If you would like to expand on your answer above you can do so below -A program of continuous review and improvement for current HTA policies and methods

Timeframes and compliance are a big downfall in all domains. Stakeholders should deliver on time in order to make treatments accessible. Language choices in reporting are also important. I've not spoken elsewhere perhaps about the fall in peer review standard and the inaccurate and fatalistic prognosis information that pervades even the newest technological approaches, by not acknowledging the efforts of clinicians for the last decade or more and worse, not giving patients an accurate foundation/information on which to base ethical decisions about affected children and their prognosis and therefore treatment choices. Critical literacy is so important and a skill for consumer representatives to consolidate - much easier if our population returned to a basic health science foundational curriculum through school. The problem is only going to worsen with Ai, aggregating from both grey and medical literature, very out of date abilities, incidence, survival rate etc.

167

In summary, considering all the draft reform options together:

How confident are you that the reform options (if implemented) will make health technology assessments better overall?

Somewhat confident

170

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

No thank you. I have not commented on too many particulars because I believe they will flow from the full collaborative partnerships between all stakeholders (with diverse expertise) but united in seeking truly transformative therapies, which will hopefully evolve to those early precision promises though we seem to be set on general designs and so expecting unclear, and barely significant statistics. The consumer risks more than any other stakeholder (for himself but moreso for those he represents in the future) and therefore must be an active critical partner in the development of effective and durable medical interventions. Oh, there are a couple words I didn't see - durable and this should be linked to a costing plan in cases where effect wanes in unison with anti-inflamatory effect or natural growth etc. Repurposing was another word I didn't see - but the subject of a separate policy draft I know.

213

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.

It was stated there was less expenditure on medicines by FN. There must be other factors waiting to be mapped here since general medicines are supplied to FN and subsidized medicines accessed at a low concession price.

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.

Critical patients should o partner in iterative decision making after long term experience/scholarship and training and it must be early as co-design/co-appraisal rather than goldilocks appealing at the end. How do we improve the literacy of stakeholders in medical publishing bias, the scientific method/bias, critical literacy, COI as younger consumers become advocates for their conditions armed with only jargon and desperation. Consider the horizon of stakeholder engagement and how that task can be made achievable. Our education system has not mandated a science to school exit for decades despite more complex understandings of health and medicine required by every consumer- a health science curriculum that taught the basics of the scientific method and critical thinking, reading and interpretation of medical literature, needs to be discussed - making the upskilling of adult health consumers easier, ensuring engagement, recruitment, treatment compliance as well as the more nuanced abilities required to analyze and make health decisions in their own treatment or about trials - albeit collaboratively with other stakeholders. Feedback loops allow consumers to use time effectively and transparently and collaboration can be enhanced by using tools to minimize power relationships such as Holacracy meeting scripts.

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.

All pathways should minimize cost and cost staggered to be linked to outcomes/certainty.

232

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.

"Draft Guidance for Industry Duchenne Muscular Dystrophy, Becker Muscular Dystrophy, and Related Dystrophinopathies '" Developing Potential Treatments for the Entire Spectrum of Disease" is an updated guidance considering the newer technologies in mind, published in recent days. These considerations for the FDA to consider and potentially adopt as guidance show the different considerations that many RD groups need in seeking approval.

See: https://content.iospress.com/articles/journal-of-neuromuscular-diseases/jnd230219

If we consider the differing perceptions of consumers in regulatory matters, eg. idebinone, Translarna are well regarded by trial families, we may be missing out on different treatments for different sub-groups eg. steroid niave, or trials that may not have run long enough to measure the benefit in a slowly progressive condition. Harmonization of approvals given by EMA or FDA even provisionally/temporarily, would allow for the generation of more data in diverse populations until higher levels of evidence have had time to be observed. Conversely, as opposed the difficulty with compliance for post-marketing approval, the time period could be shortened to revoke approval appropriately - particularly if the price negotiation was reduced until full approval given.

254

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.

International exemplar: "Draft Guidance for Industry Duchenne Muscular Dystrophy, Becker Muscular Dystrophy, and Related Dystrophinopathies '" Developing Potential Treatments for the Entire Spectrum of Disease" is an updated guidance considering the newer technologies in mind, published in recent days. These considerations for the FDA to consider and potentially adopt as guidance show the different considerations that many RD groups need in seeking approval.

See: https://content.iospress.com/articles/journal-of-neuromuscular-diseases/jnd230219 These documents do require adaptation given we have a social model of health.