| 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?** |
| Nordin Charafi |
| **7** |
| **Please select the type of individual(s) or organisation(s) you represent. Please select all that apply. - Selected Choice** |
| Pharmaceutical / Medical technology company |
| **8.1** |
| **What is the name of your organisation? - My organisation is called: - Text** |
| Illumina |
| **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 |
| **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,2.2. Proportionate appraisal pathways |
| **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)** |
| 3.1. Determination of the Population, Intervention, Comparator, Outcome,3.2. Clinical Evaluation Methods |
| **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** |
| 4.3. Understanding the performance of health technologies in practice |
| **21** |
| **Taking all Options within this section: 1.1. Transparency, communication and stakeholder involvement in HTA into account.**<br><br>**Overall, to what extent could the options (if implemented) address the issues that relate to them?** |
| Address some but not most of the issue(s) |
| **22** |
| **If you would like to expand on your answer above you can do so below:** |
| We suggest that this option to be extended to MSAC related information as well since stakeholders considered by the Reference committee pointed out language issues with MSAC processes. |
| **23.1** |
| **If implemented, overall would these Options have a positive or negative impact on you (/your organisation)? - Publish plain language summaries** |
| Neutral |
| **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** |
| Neutral |
| **27** |
| **Taking all Options within this section: 1.2. Consumer, clinician and other stakeholder engagement and consideration in HTA into account.**<br><br>**Overall, to what extent could the options (if implemented) address the issues that relate to them?** |
| Mostly address the issue(s) |
| **28** |
| **If you would like to expand on your answer above you can do so below:** |
| We suggest that this option to be extended to MSAC related information as well since stakeholders considered by the Reference committee pointed out language issues with MSAC processes. |
| **29.1** |
| **If implemented, overall would these Options have a positive or negative impact on you (/your organisation)? - Develop an engagement framework** |
| Neutral |
| **29.2** |
| **If implemented, overall would these Options have a positive or negative impact on you (/your organisation)? - Strengthen consumer evidence** |
| Neutral |
| **39** |
| **Taking all Options within this section: 1.4. State and territory government collaboration in HTA into account.**<br><br>**Overall, to what extent could the options (if implemented) address the issues that relate to them?** |
| Don't know |
| **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** |
| Neutral |
| **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** |
| Neutral |
| **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)** |
| Neutral |
| **44** |
| **If you would like to expand on your answer above you can do so below -Increase opportunities for consultation and work sharing** |
| More details are needed on the opportunities for work sharing by state and territory governments. For instance, there is a need to qualify when those collaborations will occur '“ what are 'œHTA decisions that will have significant financial and operational impact'? |
| **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)** |
| There is a need to precise which agency will have the responsibility of conducting the horizon scanning. |

**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?**

Address some but not most of 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**

Neutral

**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**

Neutral

**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**

There is a need to clarify why the unified HTA pathway would be limited to Commonwealth funding since HTA advice are not linked to funding through PBS.

**63**

**Taking all Options within this section: 2.2. Proportionate appraisal pathways into account**

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

Address some but not most of the issue(s)

**64**

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

There is a risk of deprioritizing areas with low disease burden or less costly health technologies (as compared to therapies) - particularly if we are projecting to have a unified HTA agency in the mid to long term.

**65.1**

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

Neutral

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

Neutral

**65.2**

**If implemented, overall would these Options have a positive or negative impact on you (/your organisation)? - Streamlined pathway for cost-minimisation submissions (therapies not claiming a significant improvement in health outcomes or reduction in toxicity)**

Neutral

**65.3**

**If implemented, overall would these Options have a positive or negative impact on you (/your organisation)? - Early resolution mechanisms for submissions of major new therapeutic advances in areas of HUCN:**

**Alternative option 1: Introducing an optional resolution step before HTA committee consideration**

Neutral

**65.4**

**If implemented, overall would these Options have a positive or negative impact on you (/your organisation)? - Early resolution mechanisms for submissions of major new therapeutic advances in areas of HUCN:**

**Alternative option 2: Introducing an optional resolution step before HTA committee consideration, with additional post committee resolution**

Neutral

**65.5**

**If implemented, overall would these Options have a positive or negative impact on you (/your organisation)? - Early resolution mechanisms for submissions of major new therapeutic advances in areas of HUCN:**

**Alternative option 3: Early Price negotiation**

Neutral

**65.6**

**If implemented, overall would these Options have a positive or negative impact on you (/your organisation)? - Early resolution mechanisms for submissions of major new therapeutic advances in areas of HUCN:**

**Alternative option 4: Introducing an optional resolution step after HTA committee consideration but before advice is finalised**

Neutral

**65.7**

**If implemented, overall would these Options have a positive or negative impact on you (/your organisation)? - Expanding resolution step to all relevant cost effectiveness submissions**

Neutral

**65.8**

**If implemented, overall would these Options have a positive or negative impact on you (/your organisation)? - Development of a disease specific common model (reference case) for disease areas with high active product development**

Neutral

**65.9**

**If implemented, overall would these Options have a positive or negative impact on you (/your organisation)? - Decouple the requirement for the TGA Delegate's overview to support PBAC advice**

Neutral

**77**

**Taking all Options within this section: 3.1. Determination of the Population, Intervention, Comparator, Outcome into account.**

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

Address some but not most of the issue(s)

**79.1**

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

Neutral

**79.2**

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

Neutral

**79.3**

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

Neutral

**81**

**If you would like to expand on your answer above you can do so below -Increased early stakeholder input**

We are supportive of this recommendation and we would suggest to extend its application to Genomic technologies.

**82**

**If you would like to expand on your answer above you can do so below -Increased transparency for stakeholders**

We are supportive of this recommendation and we would suggest to extend its application to Genomic technologies.

**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?**

Address some but not most of 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**

Neutral

**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**

Neutral

**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**

Neutral

**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.**

Neutral

**86.5**

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

Neutral

**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)**

Neutral

**86.7**

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

Neutral

**89**

**If you would like to expand on your answer above you can do so below -Overarching principles for adopting methods in Australian HTA**

The use of non-randomized and observational evidence, as well as RWE/ RWD for HTA purposes could be extended to other health technologies, like Genomics. The generation of RCT data are difficult for Genomic technologies, mostly because of shorter product life cycle, and therefore there is a need to recognize other sources of clinical and economic evidence to sustain the benefits of Genomics technologies going through HTA.

**94**

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

We support the development of guidelines on the assessment and appraisal of tumour agnostic therapies, should also include guideline on the assessment of associated genomic technologies. However, there is a need to better define what is meant by '˜pharmacogenomic technologies'.

Regarding the development of guidelines, the options state that this would involve '˜patients and clinicians ['¦] citizens'. There would be a need to include industry as who are instrumental in the access of Genomic technologies and can provide a breadth of expertise for the assessment of these technologies.

However, the development of guidelines will not suffice to grant better access for Genomics technologies. As pointed out in the New Frontiers report, a recommendation was made that 'œThe independent Health Technology Assessment Review reassess relevant aspects of the Health Technology Assessment process to ensure there are future pathways for treatments and therapies that do not fit neatly into the current system such as ['¦.] precision medicines.' It was further noted that 'œThe Committee is of the clear view that precision medicine approval pathways will require a different application assessment than current approaches designed for treatments for common conditions, with large data sets and comparative evaluations'. Despite the agreement from the Government, we haven't see any progress on the development of fit-for-purpose access pathways for precision medicine and Genomics technologies in particular.

**95**

**If you would like to expand on your answer above you can do so below -Pharmacogenomic technologies**

We support the development of guidelines on the assessment and appraisal of Pharmacogenomic technologies. However, there is a need to define what is meant by '˜pharmacogenomic technologies'.

Regarding the development of guidelines, the options state that this would involve '˜patients and clinicians ['¦] citizens'. There would be a need to include industry who are instrumental in the access of Genomic and can provide an expertise for the assessment of these technologies.

However, the development of guidelines will not suffice to grant better access for Genomics technologies. As pointed out in the New Frontiers report, a recommendation was made that 'œThe independent Health Technology Assessment Review reassess relevant aspects of the Health Technology Assessment process to ensure there are future pathways for treatments and therapies that do not fit neatly into the current system such as ['¦.] precision medicines.' It was further noted that 'œThe Committee is of the clear view that precision medicine approval pathways will require a different application assessment than current approaches designed for treatments for common conditions, with large data sets and comparative evaluations'. Despite the agreement from the Government, we haven't seen any progress on the development of fit-for-purpose access pathways for precision medicine and Genomics technologies in particular.

**121**

**Taking all Options within this section: 4.3. Understanding the performance of health technologies in practice into account.**

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

Mostly address the issue(s)

**122**

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

We welcome the opportunity to form advisory groups involving multiple stakeholders with the purpose of crafting guidance for the incorporation of RWE/RWD in HTA processes. We recommend that these collaborative groups specifically examine the application of RWE/RWD in evaluating Genomic technologies and emphasize the importance of including industry representatives. Recognizing the impracticality of generating traditional Randomized Controlled Trial (RCT) data for cutting-edge technologies like Genomics, we appreciate the ongoing efforts to establish appropriate pathways for utilizing alternative sources of evidence, with the active participation of all relevant stakeholders.

**123.1**

**If implemented, overall would these Options have a positive or negative impact on you (/your organisation)? - Oversight " reforms to optimise access to and use of RWD in HTA**

Neutral

**123.2**

**If implemented, overall would these Options have a positive or negative impact on you (/your organisation)? - Develop a strategic approach to increase confidence, awareness, and acceptance of cross-jurisdictional and cross-sectoral RWD access and use in HTA**

Neutral

**123.3**

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

Neutral

**123.4**

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

Neutral

**123.5**

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

Neutral

**123.6**

**If implemented, overall would these Options have a positive or negative impact on you (/your organisation)? - Collection of utilisation and outcome data for provisionally listed health technologies**

Neutral

**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?**

Not very confident

**168**

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

We cautiously welcome the proposed reform options for HTA and their potential to enhance the overall HTA landscape. Recognizing the pivotal role of Genomics in facilitating efficient diagnosis, treatment, and patient monitoring is imperative. The focus on a predictive, preventative, and personalized healthcare aligns with advancements in medical care, aiming to improve patient outcomes while optimizing healthcare system costs.

Our endorsement of reform options is accompanied by specific considerations outlined in our response to the Options Paper. We advocate for an expanded review scope of HTA's impact on health technology access, emphasizing the inclusion of Genomic technologies.

Additionally, we emphasize the need to develop guidelines for tumor-agnostic and gene therapies, incorporating associated genomic technologies. The active involvement of stakeholders, including patients, clinicians, citizens, industry representatives, and pathologists, is crucial for establishing a comprehensive and informed value framework for assessing these technologies.

Despite acknowledging the potential efficacy of guidelines, we express reservations about their adequacy in improving access to Genomic technologies. Although the government recognizes this need, progress in creating fit-for-purpose access pathways for Genomics remains elusive.

In conclusion, we appreciate the presented reform options but recognize that their effectiveness depends on thorough implementation.

**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.**

Incorporating RWE/RWD in HTA submissions can enhance the evaluation of Genomic technologies. Given the challenges in obtaining Randomized Controlled Trial (RCT) data to substantiate clinical claims for genomic technologies, there is merit in allowing greater flexibility to demonstrate their clinical and economic utility. One viable approach is to permit the utilization of RWE/RWD as a valuable means of supporting the assessment of genomic technologies.

**233.1**

**Section 2.2. of the Options Paper sets out four possible reform options relating to proportionate appraisal pathways to calibrate the level of appraisal required for HTA submissions to take the level of risk (levels of uncertainty and potential fiscal impact) and clinical need that the submission represents into account.**

**Under the subject 'Early resolution mechanisms for submissions of major new therapeutic advances in areas of HUCN', there are some options that provide different alternative mechanisms to address the issues that relate to them.**

**To what extent could the below different alternative options (if implemented) address the issues that relate to them? - Alternative option 1: Introducing an optional resolution step before HTA committee consideration**

Don't know

**233.2**

**Section 2.2. of the Options Paper sets out four possible reform options relating to proportionate appraisal pathways to calibrate the level of appraisal required for HTA submissions to take the level of risk (levels of uncertainty and potential fiscal impact) and clinical need that the submission represents into account.**

**Under the subject 'Early resolution mechanisms for submissions of major new therapeutic advances in areas of HUCN', there are some options that provide different alternative mechanisms to address the issues that relate to them.**

**To what extent could the below different alternative options (if implemented) address the issues that relate to them? - Alternative option 2: Introducing an optional resolution step before HTA committee consideration, with additional post committee resolution**

Don't know

**233.3**

**Section 2.2. of the Options Paper sets out four possible reform options relating to proportionate appraisal pathways to calibrate the level of appraisal required for HTA submissions to take the level of risk (levels of uncertainty and potential fiscal impact) and clinical need that the submission represents into account.**

**Under the subject 'Early resolution mechanisms for submissions of major new therapeutic advances in areas of HUCN', there are some options that provide different alternative mechanisms to address the issues that relate to them.**

**To what extent could the below different alternative options (if implemented) address the issues that relate to them? - Alternative option 3: Early Price negotiation**

Don't know

**233.4**

**Section 2.2. of the Options Paper sets out four possible reform options relating to proportionate appraisal pathways to calibrate the level of appraisal required for HTA submissions to take the level of risk (levels of uncertainty and potential fiscal impact) and clinical need that the submission represents into account.**

**Under the subject 'Early resolution mechanisms for submissions of major new therapeutic advances in areas of HUCN', there are some options that provide different alternative mechanisms to address the issues that relate to them.**

**To what extent could the below different alternative options (if implemented) address the issues that relate to them? - Alternative option 4: Introducing an optional resolution step after HTA committee consideration but before advice is finalised**

Don't know