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?
Wayne Massuger
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
Crohn's & Colitis 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,1.3. First Nations people involvement and consideration 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,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
5.1. Proactively addressing areas of unmet clinical need and gaps in the PBS,5.5. Capacity and capability of the HTA system
25
If you would like to expand on your answer above you can do so below -Publish plain language summaries
CCA support this option to improve consumer access to understand what the application is seeking and enable them to provide relevant feedback. Current one line PBAC Agenda listings do not provide sufficient information for consumers to understand what is being sought through the application e.g. is it a novel agent with a different therapeutic action, is it a different preparation with administration differences.
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
CCA support improvements to the HTA website and introduction of a dashboard. In particular web information that simplifies and clarifies the real-time progress of drugs through the HTA system is a priority
31
If you would like to expand on your answer above you can do so below -Develop an engagement framework
We support the involvement of consumers, clinicians and other relevant stakeholders in an engagement framework - this will help ensure the actual problems and patient populations are being addressed through shared decision making and discussion. There is an over reliance on RCTs that can be rigid and artificial, far removed from the reality of clinical encounters faced by the majority of people with the condition outside of RCTs .
32
If you would like to expand on your answer above you can do so below -Strengthen consumer evidence
We support use of consumer evidence, particularly around the use of Real-World-Evidence, patient reported experience measures and equity. This data is already available for IBD through data sources such as Crohn's and Colitis Care and the CCA national IBD audits. This is critical to help change the assessment criteria for new therapies that are hamstrung by not having done multiple RCTs against all available therapies to show superiority where we have a large group of patients who have lost response to all available therapies and are hence left with no advanced therapy option. The reliance on RCTs for every approval is increasingly unrealistic and unaffordable (especially the placebo- controlled and head to head against multiple therapies) and leave many without new options after lost response. There should be a focus on evaluation over a multi-year timeframe and must include indirect costs - especially time out of work/role.
37
If you would like to expand on your answer above you can do so below -First Nations peoples partnership in decision making
CCA supports the inclusion of a First Nations people partnership in decision making. Though prevalence of IBD in First Nations people is thought to be low, CCA recognises the health disadvantage of this group and hence the importance of their input to improve engagement in HTA.
50
Pathway for drugs for ultra-rare diseases (Life Saving Drugs Program (LSDP))
CCA supports these options to improve the process and reduce duplication for LSDP. IBD is not an ultra-rare disease, however there is a paediatric subgroup of very young children with IBD (Very Early Onset '" VEO-IBD). They are small group with high morbidity, and some mortality who would benefit from access to advanced therapies that have no other path to access.
60
If you would like to expand on your answer above you can do so below -Vaccine pathway
CCA supports the expanding the role of PBAC for a broader range of health technologies including co-dependent health technologies. This will be critical for emerging cell-based therapies and potentially gene editing for monogenic causes of chronic inflammatory diseases. Cell based therapies are not 'pharmaceutical' and have no clear funding pathway.
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
CCA support the unified, national, HTA pathway for all health technology evaluation that will draw on appropriate specialists for all advanced therapies and technologies seeking public funding and being able to recommend the appropriate funding pathway.
68
If you would like to expand on your answer above you can do so below -Streamlined pathway for cost-minimisation submissions (therapies not claiming a significant improvement in health outcomes or reduction in toxicity)
CCA support streamlining pathway for cost-minimisation submissions to avoid delay in gaining access to medications. It is important to ensure cost minimisation approach is not applied to new therapies if they are actually novel.
70
If you would like to expand on your answer above you can do so below -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**
CCA support this as it may allow access to therapies for indications that currently do not have the high level of phase 3 registration clinical data and where these trials are very unlikely to occur due to commercial decisions e.g. fistulising disease where only luminal disease has been adequately assessed. Appropriate guidelines may encourage funding of targeted local trials to assess specific efficacy and enable funding. Data should be gathered in real world care for subsequent review and if data do not show value, access will be curtailed.
71
If you would like to expand on your answer above you can do so below -Early resolution mechanisms for submissions of major new therapeutic advances in areas of HUCN **Alternative option 3: Early Price negotiation**
CCA support earlier price negotiation in the process - the current situation of positive PBAC recommendation to then have a non-sustainable cost offered is disheartening for patients and clinicians and a huge waste of resources.
74
If you would like to expand on your answer above you can do so below -Development of a disease specific common model (reference case) for disease areas with high active product development
There is some concern that this risks examination of the use of medications for alternative disease groups, thereby slowing access to medications across a wider disease profile.
81
If you would like to expand on your answer above you can do so below -Increased early stakeholder input
CCA support increased early input on the PICO from patient and clinician communities to ensure all relevant patient populations that could potentially benefit from the new therapy are considered in the HTA, and to identify issues that may impact implementation early to be addressed (for new drugs or major expanded indications claiming added therapeutic value).
83
If you would like to expand on your answer above you can do so below -Updated guidance
CCA support updated guidance to require the explicit consideration of health equity and priority populations for new treatments. These populations should specifically include patient population that can no longer benefit from the comparator classes of drugs due to either contraindications or prior loss of response. This would allow subgroup analyses of for instance anti-TNF experienced patients in trials. CCA Support the ability to use real world evidence in submission because sponsors will never do head-to-head comparisons of new agents against all agents in class, but post marketing evidence from clinicians is highly influential to practice and should be represented in the funding decisions.
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
Support the ability to use real world evidence in submissions broadly and not on a restricted basis because sponsors will never do head-to-head comparisons of new agents against all agents in class, but post marketing evidence from clinicians is highly influential to clinician practice and should be represented in the funding decisions. This is important for paediatric IBD patients who need access to medications approved for adults but lack research evidence in the paediatric population. RWE is often available in Australia or in countries with well-developed health systems and should be used to support access to t medications that provide important alternative for those who have failed other treatment options. Similarly, inclusion of evidence about elderly populations , who are rarely included in IBD RCTs, is required to support quality use of medicines in this group. Currently PBS criteria restrict dosing of biological therapies. CCA supports the use of RWE to inform flexible dosing whee there is a clinical benefit to the patient. Responsive systems are required to translate evidence into change in access to drugs.
93
If you would like to expand on your answer above you can do so below -Develop an explicit qualitative value framework
In addition to just looking at cost effectiveness across a trial population, look at the health economic modelling of the patient groups that have a higher clinical need or fewer available therapies or are excluded from clinical trials - the socioeconomic effects of failure to list a new therapeutic class are far greater on these patients than on treatment naive patients that make up to bulk of the registration studies for earlier therapies that are now used as the reference comparator. Indirect costs like time out of work/role should be included in the health economic modelling.
94
Therapies that target biomarkers (e.g. tumour agnostic cancer therapies, therapies that target particular gene alterations)
There is hesitancy among the community to have compulsory genetic testing linked to access to drugs. This is primarily related to privacy of genetic information and risk of disclosure.
100
If you would like to expand on your answer above you can do so below -Selection of the comparator
CCA support the development of guidelines to distinguish between the selection of comparator for submissions claiming superiority and to submissions claiming non-inferiority to make clear which comparator should be selected when there are multiple potential comparators. These groups should include patients who have failed prior therapies that may be being used as the comparator and hence are not clinical options and invalid. Comparisons need to be fair, transparent and realistic. There is also support for listing multiple non-inferior drugs with price equity to minimise supply chain risk that has plagued some medications in IBD over recent years.
102
If you would like to expand on your answer above you can do so below -Valuing overall

CCA is concerned that the current lowest price comparator model and subsequent price reductions is resulting in newer therapies that can even be first in class not being brought to Australia even when given a positive recommendation by the PBAC. Adapting the comparator and criteria for demonstrating superiority needs to be changed to reflect the actual benefit to the patients - including those with not currently available effective therapy due to prior loss of response or intolerance to all other available classes.
Australia risks being seen internationally as an unattractive place to bring new therapies or even involve in clinical trials of new therapies due to the increasingly unlikely possibility of a price that represents actual cost of development and manufacture. This is counter to the goal of increasing access for Australians to best therapies.

108

If you would like to expand on your answer above you can do so below -Recognising competition between new health technologies that deliver similar outcomes:

Alternative option 2: In conjunction with options for proportionate assessment of cost-minimisation submissions, incentivise offers of a lower price for health technologies that provide no added benefit

CCA support choice in medications even if they deliver similar outcomes. New or existing medications that offer similar outcomes in aggregate may be more, or less effective for an individual. This is commonly reported in IBD and choice in therapeutic action and even in preparation are important differentials for individual response.

112

If you would like to expand on your answer above you can do so below -Approaches for managing uncertainty - bridging funding coverage for earlier access to therapies of likely HATV and HUCN

CCA support choice in medications even if they deliver similar outcomes. New or existing medications that offer similar outcomes in aggregate may be more, or less effective for an individual. This is commonly reported in IBD and choice in therapeutic action and even in preparation are important differentials for individual response.

161

If you would like to expand on your answer above you can do so below -Improve HTA capacity and workforce in Australia

CCA support sponsored internships and we suggest that these are targeted to be inclusive of a range of cultural and minority groups to ensure promote diversity across the HTA system.

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

The consultation 2 draft Options paper is a technical document of 175 pages which is not a suitable format for direct distribution for people living with chronic health problems or other health conditions. The taks can seem overwhelming for people living with a health condition who may experience a degree of fatigue or limited concentration. CCA request future consultation docment be provided with a more brief and simplified version designe with consumer to optimise the likelihood of high quality feedback.