Medicines Australia Submission

Public Consultation – Revised Procedure Guidance – Public Summary Documents

Background:

Medicines Australia is supportive of the Pharmaceutical Benefits Advisory Committee's (PBAC's) objective for the publishing of Public Summary Documents (PSDs) to be efficient, consistent, and reflective of the committee's decision-making deliberations.

Medicines Australia also acknowledges the level of engagement & consultation afforded industry on these important proposed changes to the PSD processes and the PSD redaction criteria. Medicines Australia is pleased that initial informal discussions through the first half of 2019, together with official, broader consultation in August 2019, has led to an evolution of the proposed changes to process and redaction criteria, such that:

- ***Implementation of proposed changes has been moved from July 2019 to July 2020*** – this allows applicants to fully understand, communicate with global colleagues and prepare for implementation. This also allowed time for a pilot process to consider the impact of the changes on existing submissions with feedback from the pilot process used to inform the final process and criteria for PSDs.
- ***Implementation of process and redaction changes will be aligned*** – the movement of process changes from Nov 2019 to July 2020 & redaction criteria changes from March 2020 to July 2020 will allow for change management alignment and significantly reduce the potential for applicant confusion.
- ***An appeals process has been included*** – this will provide the applicant greater confidence that procedural fairness is available prior to PSD publication.
- ***A qualitative summary will replace redacted clinical data*** – this will allow the reader to be more informed with regards to the PBAC's decision making process and rationale.
- ***Redaction of clinical data is allowable, should it be shown to be linked to the potential back-calculation of confidential pricing*** – this will provide the applicant with greater confidence that pricing information will continue to be adequately protected.
- ***Redaction of clinical data is allowable, should it potentially breach study participant confidentiality*** - this will provide the applicant with greater confidence that important patient related information is kept private/ confidential.

Remaining Concerns:

Despite significant improvement in the proposed changes to PSD process and redaction criteria over the course of 2019, Medicines Australia remains extremely concerned that the clinical data redaction criteria are still too narrow and may have the adverse impact of applicants not including all potentially relevant data in PBAC submissions. Additionally, applicants may not be able to take advantage of the parallel process and/or be significantly de-prioritised by the global organisation as a launch market if these proposed changes were implemented and confidentiality of data was not assured. Medicines Australia also notes that significant administrative burden for applicants (both locally and globally) is associated with the proposed redaction criteria and processes.

Medicines Australia has listed specific examples in Table 1, demonstrating the circumstances in which applicants will potentially not include relevant clinical data within their PBAC submissions should the proposed redaction criteria remain as proposed.

Table 1: Examples of clinical data that may not be provided in future PBAC submissions or lead to significant delay in submitting applications to the PBAC

Examples of data which may not be included in PBAC submissions because they don't fit the proposed redaction criteria	***Reason***
Patient reported outcomes from key clinical studies that have not been submitted for publication at time of PBAC submission.	• Often, patient reported outcomes (PROs) are classified in clinical trials as secondary or subsequent endpoints and not part of the primary journal publication. • Medicines Australia believes that this situation is not covered in the proposed clinical data redaction criteria, and as such, parent companies may reject Australian affiliate requests for inclusion of PRO data in a PBAC submission. • Medicines Australia would support the use of a qualitative statement with the redaction of unpublished PRO clinical data, such that the PBAC decision making process remains transparent.
Indirect treatment comparison (ITC) specific to comparators in the Australian healthcare system, using unpublished data.	• Rarely are these ITC data analyses published, and for the small percentage that are, they are usually published in a journal well after PBAC consideration. • Medicines Australia believes that this situation is not covered in the proposed clinical data redaction criteria, and as such, parent companies may reject Australian affiliate requests for inclusion of clinical data in a PBAC submission for fear that an ITC will be conducted and published using unpublished data. • Medicines Australia would support the use of a qualitative statement with the redaction of unpublished clinical data used in the construction of an ITC, such that the PBAC decision making process remains transparent.
The applicant is not responsible for relevant clinical trial data – e.g. a clinical organisation or independent clinical investigator may be running the relevant clinical study.	• In this situation, applicants are unlikely to know, or be able to find out, the publication plans and status of any manuscripts that the clinical organisation may be working on. • Medicines Australia believes that this situation is not covered in the proposed clinical data redaction criteria, and as such, third-party owners of data may reject the applicant's request for inclusion of third-party data in a PBAC submission. • Medicines Australia would support the use of a qualitative statement with the redaction of third-party data used in PBAC submissions, such that the PBAC decision making process remains transparent.
Data may only be published in a conference poster or oral presentation but not in a manuscript (or not in a manuscript for a long time).	• Medicines Australia believes that this situation is not covered in the proposed clinical data redaction criteria, and as such, parent companies may reject Australian affiliate requests for inclusion of clinical data from conferences. • Medicines Australia would support the use of a qualitative statement with the redaction of unpublished conference data used in PBAC submissions, such that the PBAC decision making process remains transparent.

Post-hoc subset data analyses specific to the Australian HTA system and/or in response to the Evaluator or PBAC requests.	• Rarely are these post-hoc subset data analyses published, as they are often not considered statistically robust. Publication of these exploratory results would not be accepted or suitable under the journal peer-review process, and could be reputationally damaging for applicants and/or investigators. For the small percentage that are, they are usually published in a journal well after PBAC consideration, having typically been developed during the submission development phase or while the submission was under evaluation. • Medicines Australia believes that this situation is not covered in the proposed clinical data redaction criteria, and as such, parent companies are likely to reject the local applicants request for inclusion of post-hoc subset data in PBAC submissions. • Medicines Australia would support the use of a qualitative statement with the redaction of post-hoc subset data used in PBAC submissions, such that the PBAC decision making process remains transparent. • To provide context for readers who may be less familiar with the PBAC submission process (e.g. other markets, prescribers & patients), Medicines Australia believes it is important for applicant companies to be able to present their own interpretation of these post-hoc data subsets, including a validation test of data-cuts, to show the statistical relevance of any information.
Post-hoc data analyses (e.g. subgroup analysis) conducted by the Evaluator and/or Department during the evaluation process.	• These post-hoc data analyses are not published. • Medicines Australia believes that this situation is not covered in the proposed clinical data redaction criteria, with parent companies in the past requesting redaction of this data on the basis of not wanting post-hoc data analyses that are specific to the Australian HTA system and conducted by parties other than the applicant, being published and potentially causing confusion or commercial risk in other jurisdictions. • Medicines Australia would support the use of a qualitative statement with the redaction of post-hoc data analyses used in Evaluations, such that the PBAC decision making process remains transparent.
Unpublished safety data	• It is possible that the PBAC will see/review emerging safety data prior to the Regulator reviewing/taking a position on the data. • Medicines Australia believes that this situation is not covered in the proposed clinical data redaction criteria, with applicants being extremely concerned that publication in PSDs could unduly influence TGA decision making. • Medicines Australia would support the use of a qualitative statement, such that the PBAC decision making process remains transparent.

Medicines Australia's Recommendations:

Medicines Australia proposes that:

- Key learnings should be shared from the pilot process that is underway, including a high-level summary of how any issues relating to the above examples were addressed throughout this process. Ideally, this would occur ahead of the implementation in July 2020 and would also be used to refine the PSD redaction criteria and process outlined in the Procedure Guidance without delay.
- Due consideration be given to the examples in Table 1 and the proposed redaction criteria be adjusted accordingly. For example:

    - the replacement of redacted clinical data with a qualitative statement will allow for transparency of the basis for PBAC decision making whilst appropriately maintaining confidentiality of unpublished clinical data.
    - the allowance of a footnote specific to Australian relevant bespoke analysis that may not have been pre-specified in the original clinical trial statistical plan – with the footnote providing context around the relevance to PBAC decision making and the applicant's clinical interpretation of the bespoke analysis.

- Until it has been established that, following implementation of the new criteria, confidential prices cannot be back-calculated, Medicines Australia proposes that the existing ICER and budget impact ranges be maintained. The timing for implementation of changes to published ICER and budget impact ranges should be agreed with Medicines Australia.
- Greater transparency with regards to PBAC decision making in PSDs be extended to include further detail to assist readers in understanding the basis for each PBAC determination of interchangeability.
- Greater transparency with regards to PBAC decision making in PSDs be extended to include further detail to assist readers in understanding the clinical expert advice that has been sought by PBAC and how that advice has informed the PBAC recommendation.
- A joint Department of Health / Medicines Australia working group be established to monitor the pilot process and implementation. The redaction criteria, process and associated Procedure Guidance should then be amended as appropriate, without delay.

Medicines Australia has also provided ***Track Change*** edits and comments specific to the PSD Guidance Document and tenders this alongside this submission. Edits and comments within the PSD Guidance Document are minor in nature.

Conclusion:

Medicines Australia supports the view that transparency of decision making is in general helpful, and must be conducted in a way that improves outcomes for all stakeholders. However, Medicines Australia believes that the current proposed clinical data redaction criteria will lead to either significant omissions of unpublished clinical data from PBAC submissions or potential delayed lodgement of submissions. Australian affiliates will not submit analyses as their parent companies will not permit publishing of confidential clinical data. This will lower the level of relevant information for decision making, which, in turn, may lead to an increased risk of initial submissions being delayed, greater uncertainty for the PBAC in their deliberations if important data are missing, increased rejections, submission churn and resultant delays to Australian patients accessing new medicines via the PBS. All stakeholders should anticipate detrimental effects to the health care system and delayed patient access to innovative therapies if all clinical data is made transparent, rather than working to balance the needs and interests of all parties.

Medicines Australia re-iterates its thanks for the open and constructive dialogue on this topic through 2019 and requests the Department/ PBAC to continue to engage to ensure the changes benefit all stakeholders.