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Dear Sir/Madam

Consultations on PBS Process Improvements

Medicines Australia welcomes the opportunity to provide comment on the Streamlined Pathways consultations on PBS Process Improvements. This feedback has been consolidated based on input from the Medicines Australia representatives on the Streamlined Pathways Subgroup of the Access to Medicines Working Group (AMWG) and the Health Economics Working Group.

This submission is lodged without prejudice and subject to any further advice we receive.

CONSULTATION ON THE PRE-SUBMISSION BRIEFING: Procedure Guidance and Forms

Medicines Australia supports the improvements to the pre-submission meeting process, especially with doubling of access to early advice for complex submissions and ensuring there is a shared understanding of the meeting outcomes.

The major recommendations are:

- In order to improve the quality of pre-submission meetings in general, it would be useful for the procedure guidance and forms to focus on positive language, and 'what good looks like' rather than what it doesn't look like (see example under 'Pre-submission Briefing Document Form' below).
- Revise the Pre-Submission Meeting Request to include key questions to be raised and the removal of the proposed details in the PBAC submission (e.g. proposed PBS restriction, main comparator, clinical claim, economic claim) which will be addressed in the briefing document.
- Ensuring it is clear the sponsor is not required to complete all areas of the pre-submission briefing document but to provide enough information to gain the required advice.
- Allow applicant agreement to provide de-identified feedback to Medicines Australia to support ongoing meeting improvements.
- Removal of the applicant declaration that is currently included in the forms. The enhanced pre-submission process is to encourage solution-focused, constructive discussion. It is not in the sponsor's best interest to present false or misleading information given the subsequent submission is based on the findings documented in Clinical Study Reports. We would imagine it would always be complete and correct to the best of the author's knowledge. It is also unclear who the authorised person would be, or when a fee would not be payable. This declaration gives the impression that the document is a contractual arrangement.

Pre-Submission Meeting Guidance:

Medicines Australia welcomes the guidance for the pre-submission process, focusing on providing consistency and supporting best practice to deliver the required advice to support optimal submission development. However, there is significant concern that the timelines proposed are not optimal nor aligned to current pre-submission timelines and Medicines Australia proposes a change in both the length of time to confirm the meetings and for finalisation of the meeting outcomes.

The Proposed changes to the guidance and timing of the meetings are outlined below:

- ***Rejection of a meeting must be done in consultation with the applicant****:* As there are clear criteria for pre-submission meetings, it is important a sponsor has the opportunity to seek early advice on the submission. If the Department declines the opportunity, it must be in consultation and agreement with the applicant. This important consideration must also be captured in the Cost Recovery Regulations.
- ***Timing of the first meeting:*** Medicines Australia acknowledges the preference for the first meeting to be at least 4 months (16 weeks) prior to the planned PBAC submission date; however, requests that the sponsor has the ability to have the first (and only) meeting a minimum of 2 months (8 weeks) prior to the planned PBAC submission date.
- ***Timing of Department response to pre-submission meeting request:*** If the Department responds to the pre-submission meeting request at Day 14, there is no time for sponsor to submit the pre-submission briefing document. This briefing document would need to be lodged immediately, on the same day, in order to be received 14 days before the pre-submission meeting. It is requested that the Department's responds to the sponsor by Day 5 of receiving the meeting request so the sponsor is able to prepare and submit the briefing document on time.
- ***Duration of pre-submission meetings scheduled for up to 90 minutes:*** One hour may be inadequate for complex applications based on current sponsor experiences. Given cost recovery fees will be in place, an extension to 90 minutes is requested (where needed) to address the key questions and the briefing material provided. The extension of time may also be considered based on the different representatives at the meeting - for example, MSAC, TGA.

Overall timeline of pre-submission meetings

Medicines Australia has taken the opportunity to undertake a holistic review of the pre-submission meeting timelines against proposed transitional timelines (issues to the SPS working group prior to the consultation). The current procedural guidance does not match with the proposed timelines and furthermore the proposed response times does not provide an optimal engagement allowing for process improvement.

Please see **Table 1** for the current proposed timelines as per the transitional timeline versus a backward calculation from the PBAC major submission deadline to meet the statements made in the procedural guidance. It is noted full date ranges are not included but rather a single date is included for illustrative purposes.

The table highlights:

- The need to re-align the pre-submission dates to be available at least 8 weeks prior to the major PBAC deadline.
- The need to change the 14-day confirmation of the meeting as it does not allow time to prepare and submit the briefing document.

- The need to change confirmation of meeting outcomes from both parties from 14 days to 5 days. While it is recognised this a significantly reduced time, it is important to ensure the process can continue.

Table 1: Proposed Transitional Timelines versus Procedural Guidance

Week	Activity	PBAC Cycle 2020/1 Meeting Date: March 2020	Procedural Guidance
-20	1st Pre-Submission Meeting Request	15/07/2019	19/06/2019
-19			
-18	Department confirm Pre-Submission Meeting		3/07/2019
	Deadline for pre-submission Briefing Document	29-July - 23 Aug 2019	3/07/2019
-17	Confirmation of attendees (2 days prior to meeting)		15/07/2019
-16	**Pre-Submission Meeting 1 (must held minimum 16 weeks before submission)**	12-Aug-6-Sept 2019	**17/07/2019**
-15			
-14	Sponsor provides Meeting Outcomes		31/07/2019
-13			
-12	Department confirms Meeting Outcomes		14/08/2019
	2nd Pre-Submission Meeting Request	26/08/2019	14/08/2019
-11			
-10	Sponsor provide Pre-Submission Briefing document	9-13-Sept 2019	28/08/2019
	Department confirm Pre-Submission Meeting		28/08/2019
-9	Confirmation of attendees (2 days prior to meeting)		9/09/2019
-8	**Pre-Submission Meeting 2 (must held minimum 8 weeks before submission)**	23-27-Sept 2019	**11/09/2019**
-7			
-6	Sponsor provides Meeting Outcomes		25/09/2019
-5			
-4	Department confirms Meeting Outcomes		9/10/2019
	Deadline for Intent to Apply Form for Major submissions	9/10/2019	9/10/2019
-3			
-2			
-1			
0	Deadline for major submissions	Nov-19	Nov-19

To address the timing issue, Medicines Australia proposes **Table 2**, that alters the timing of the following process:

- Change the 14-day confirmation of the meeting to 5 days, allowing time for the applicant to prepare and submit the briefing document
- Change meeting outcomes from both parties from 14 days to 5. While it is recognised this a significantly reduced time, it is important to ensure the process can continue, and there is adequate time to complete and submit the Intent to Apply form.

Table 2: Medicines Australia proposed timeline

Week	Activity	Medicines Australia Proposal
-20	1st Pre-Submission Meeting Request	19/06/2019
-19	Department confirm Pre-Submission Meeting (5 days)	26/06/2019
-18	Deadline for pre-submission Briefing Document	3/07/2019
-17	Confirmation of attendees (2 days prior to meeting)	15/07/2019
-16	**Pre-Submission Meeting 1 (must held minimum 16 weeks before submission)**	**17/07/2019**
-15	Sponsor provides Meeting Outcomes (5 days)	24/07/2019
-14	Department confirms Meeting Outcomes (5 days)	31/07/2019
-13		
-12	2nd Pre-Submission Meeting Request	14/08/2019
-11	Department confirm Pre-Submission Meeting (5 days)	21/08/2019
-10	Sponsor provide Pre-Submission Briefing document	28/08/2019
-9	Confirmation of attendees (2 days prior to meeting)	9/09/2019
-8	**Pre-Submission Meeting 2 (must held minimum 8 weeks before submission)**	**11/09/2019**
-7	Sponsor provides Meeting Outcomes (5 days)	18/09/2019
-6	Department confirms Meeting Outcomes (5 days)	25/09/2019
-5		
-4	Deadline for Intent to Apply Form for Major submissions	9/10/2019
-3		
-2		
-1		
0	Deadline for major submissions	Nov-19

Pre-Submission Meeting Request Form:

Medicines Australia understands the need for consistency in preparing for a PBAC submission and ultimately PBAC consideration, however it is important to distinguish there is a different level of information available between requesting a pre-submission meeting and the information collected for the purposes of the Intent to Apply form. The forms need to be significantly different.

The purpose of the pre-submission meeting is to access early advice from Department of Health (or approved external experts) to support the applicant in the development of the submission. The meeting request should provide enough information to indicate the medicine to be discussed and the key questions that are intended to be raised. As such the form should include the TGA status and proposed indication and remove any details about the proposed PBAC submission details – these details will be included in the briefing document and highly likely to be subject to the discussion.

Pre-Submission Briefing Document Form:

Medicines Australia requests the introduction of the briefing document clearly stipulates that the purpose is to provide sufficient information to gain the required advice to support the PBAC submission. The timing of the pre-submission meeting will occur at different points in the development of the submission and not all information will be available at time of submission, especially pricing.

Given the purpose of pre-submission meetings is to receive non-binding advice, it is requested that the Department includes example questions for discussion at the meeting, as a guide to ensure best practice. In order to improve the quality of pre-submission meetings, it would be useful to focus on what good

looks like rather than what it doesn't look like. For example: "Questions for the Department should help gain a mutual understanding around reasonable approaches, claims or modelling, for example…." rather than "Questions for the Department should not include requests to confirm/agree approaches, claims or modelling".

Pre-Submission Meeting Outcome Form:

Positive or useful points of engagement should be captured to record what worked well at the meeting. This will lead to a collection of best practice and improve the quality of the pre-submission process.

Medicines Australia requests de-identified meeting feedback to be captured and be provided in a timely manner to enable engagement with the Department of Health on best practice and where future improvements can occur. As Medicines Australia represents the discovery-driven pharmaceutical industry the feedback is important for continuous improvement.

As the meeting outcomes form is a concise and complete document of the meeting, and can be shared as part of the PBAC submission process, the meeting outcome needs to capture attendees.

CONSULTATION ON THE INTENT TO APPLY: Procedure Guidance and Form

Medicines Australia supports the Intent to Apply process, with modifications to the procedure guidance and the form to assist the planning process both from the applicant and the Department of Health.

The major recommendations are:

- Consistency between the Guidance and the Cost Recovery Regulations;
- Inclusion of amendment and postponement to the Intent to Apply form;
- Removal of the proposed comparator from the Intent to Apply form;
- Consistent language and data collected between the TGA and PBAC process, especially given the proposal of greater transparency for healthcare consumers;
- Removal of the applicant declaration that is currently included in the form.

Intent to Apply Procedure Guidance

Medicines Australia welcomes the guidance for the Intent to Apply process but requests consistency between the proposed documents and the Cost Recovery Regulations. Medicines Australia notes under the Cost Recovery Regulations there will be an exemption of the process if a submission is required to address an urgent public health need and this is not currently documented in the Procedure Guidance.

Medicines Australia also recommends formal communication of the final guidance documents given the significance of the process to the assessment of medicines. All pending applicants need to be aware that failure to submit an Intent to Apply form 28 working days prior to the PBAC major/minor submission deadline will result in the submission not being considered at the proposed PBAC meeting. This is a significant change from the current process and Medicines Australia suggests the PBAC Calendar and other guidance documents outlining the submission process are updated to include these deadlines before July 2019.

Medicines Australia further seeks to undertake a joint review of the Intent to Apply process prior to making any amendments prior to the introduction of the proposed Stage 2 PBS process improvement changes from 1 July 2020.

Proposed changes to the guidance, outside of a formal reference to the Cost Recovery Regulations, are outlined below:

- **Automatic confirmation is required**: Given the significance of the Intent to Apply form, it is important that an email acknowledgement of receipt will be immediate.
- **Amendment, postponement and withdrawal:** Significant interactions with global affiliates in the lead up to PBAC submission deadlines means there can be changes in the last 4 weeks prior to finalisation and submission of a PBAC application. As such, Medicines Australia seeks to restore the process on amendment and postponement to the Intent to Apply form.
  - **Amendment**: Sponsors may contact the Department to amend the details provided in their Intent to Apply form within the 4-week period without incurring any additional fee.
  - **Postponement**: Within the 4-week period following lodgement of the Intent to Apply form, sponsors may notify the Department to postpone their submission for the upcoming PBAC meeting, to instead be considered at the next meeting, without incurring any additional fee.
- **Transitional arrangements:** Medicines Australia acknowledges the Department has requested sponsors of major submissions for the November 2019 PBAC meeting will have the option to lodge an Intent to Apply form in June 2019 for the July PBAC cut-off. As part of this option it needs to be explicit that a sponsor who does not submit an Intent to Apply in June, but a Major submission by the 10th July 2019 it will be considered at the November 2019 PBAC meeting.

Intent to Apply Form:

The purpose of the Intent to Apply form is to support adaptive resourcing and efficient processes of the Department of Health to manage the volume and type of submissions requiring evaluation. Medicines Australia believes the identification of the medicine, disease area, proposed PBS restriction, clinical & economic claim, as well as whether the application will refer to a potential Managed Access Program/ Managed Entry Scheme, are all relevant for potential planning and resourcing purposes. However, Medicines Australia does not believe that the identification of the main comparator adds any value to resource planning and recommends it be deleted from the form.

The following are additional recommended changes to Intent to Apply form, and for ease of reference are highlighted on the form:

- ***Applicant (previously sponsor):*** Medicines Australia is aligned with the change in the terminology from sponsor to applicant. The change in wording recognises a PBAC submission may be submitted by different parties and the applicant may not be the sponsor of the medicine.
- ***Application date***: Recommended there is space for the applicant to add a date that corresponds to the submission of the Intent to Apply form.
- ***Medicines /vaccines name****:* To ensure consistency between applications, Medicines Australia recommends to include two separate fields to capture both drug name and trade name (similar to what is currently captured in the PBAC agenda).
- ***Therapeutic Area:*** As part of the TGA transparency consultation on "whether the TGA should publish that a prescription medicine is under evaluation", Medicines Australia supports the Therapeutic Area to be published. Medicines Australia recommends the ATC therapeutic sub-group being used as a consistent reference, with the same information being captured for reproduction on the PBAC agenda.
- ***Deadline for Major/Minor submission*****:** To align with the current PBAC calendar and as part of the transition period (2019/2020), it is recommended the wording of PBAC Submission Date be changed to Deadline for major / minor submission. The may change with the introduction of different submissions or pathways post July 2020.

- ***Proposed PBS Restriction**:* For ease and consistency applied to all applications, Medicines Australia recommends to add a drop-down box of PBS restriction types – for example, Unrestricted, Restricted, Authority Required, Authority Required (STREAMLINE)
- ***Managed Entry Scheme (MES) / Managed Access Program (MAP**)*: The potential for a MES/MAP may evolve across the course of the evaluation. As such, Medicines Australia recommends addition of a to be determined (TBD) category to be included in the application. Medicines Australia also recommends that the MES/MAP query be moved to below the clinical and economic claim sections.
- ***Removal of proposed main comparator:*** As raised above, Medicines Australia request the proposed main comparator be removed as such identification does not add any value to resource planning.
- ***Clarification on "are there any concurrent (or intended) applications in process?"*** Medicines Australia is not clear on the intent of the question and have attempted to provide clarity by changing the word to be "are there any concurrent applications in process for the same molecule?" Further clarity on the intent of the questions will be required to ensure the applicant provides clear information on the form – for example, does this refer to a concurrent MSAC application for a co-dependent technology? If this is not the intent, we welcome further insight as part of our ongoing dialogue.
- ***Removal of applicant declaration**:* It is requested that the applicant declarations be removed from all of the forms. It is not in the sponsor's best interest to present false or misleading information given the subsequent submission is based on the findings documented in Clinical Study Reports. Medicines Australia would imagine it would always be complete and correct to the best of the author's knowledge. It is also unclear who the authorised person would be, or when a fee would not be payable. This declaration gives the impression that the document is a contractual arrangement.

Yours sincerely,

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