OTHER MATTERS

Managed Access Program Framework (Agenda item 11.03)

The PBAC reviewed the '*Draft Framework for the Managed Access Programme for submissions to the PBAC*' (dated 11 March 2015), that was prepared by the Managed Access Programme (MAP) Sub-Group of the Access to Medicines Working Group.

1 Purpose of Item

1.1 To provide the PBAC with the opportunity to comment on a draft of the *'Framework for the Managed Access Programme for submissions to the PBAC'* currently being considered and revised by the Department of Health and Medicines Australia through the Access to Medicines Working Group (AMWG).

2 Background

2.1 Under clauses 26 and 27 of the Memorandum of Understanding (MoU) between the Department of Health and Medicines Australia, which expired in June 2014, the Managed Entry Scheme (MES) was introduced to enhance the quality and strength of evidence provided to decision-makers in reimbursement applications and allow the PBAC to recommend PBS coverage at a price justified by the existing evidence, pending submission of more conclusive evidence of cost-effectiveness to support the listing of the drug at a higher price.

2.2 Under the MoU it was agreed that the application of this mechanism was initially to be restricted to submissions where the PBAC agreed that there was a clinical need for the intervention, and when:

- the PBAC would not otherwise recommend the listing of the drug at the proposed price because the extent of the clinical effect was uncertain and/or there is a high cost to the PBS overall and/or on a per patient basis; and
- there was evidence that can credibly report within a reasonable timeframe, which the PBAC was satisfied will resolve the identified area of uncertainty.

2.3 During the period of the MoU there have been several changes in approach to managing uncertainty and risk arising from submissions for PBS subsidy of medicines considered and recommended by the PBAC, and through the experience of drugs that have been through the MES.

2.4 A new Framework for a Managed Access Programme (MAP) is currently being developed by the Department and Medicines Australia through the MAP Sub-Group of the AMWG.

3 PBAC Discussion

3.1 The Committee provided the following comments on the draft document.

Over-arching comments

- The MAP process should be reserved for therapies for which:
    - there is a high and urgent clinical need;
    - based on the data available, the PBAC believes there will be a significant net benefit from the therapy, but is uncertain about the magnitude of that benefit and therefore the price that should be paid; and
    - there is a strong expectation that data will be forthcoming over a reasonable time period and this data should address the areas of uncertainty.
- A broader consultation on the MAP framework may be required to ensure that it reflects community values.

PBAC involvement in the MAP process

- Significant resources are required to fully evaluate a MAP proposal, substantially more than those required for evaluation of a typical major submission.
- Should MAP proposals arise during the evaluation period (eg. as part of a Pre-PBAC or Pre-Sub-Committee Response), an extended timeframe for consideration of the submission would be required to enable adequate evaluation.
- In addition, a systematic, independent and rigorous evaluation process would be needed to resolve areas of clinical and/or economic uncertainty if the PBAC recommends a drug for listing within the MAP parameters. The details of this evaluation process, including planning for expected outcomes, needs to be agreed to by all the parties prior to listing of a medicine on the PBS under a MAP.
- In addition to its central role in defining each MAP, the PBAC should also have a central role in reviewing the subsequent evidence generated to address the identified issues of uncertainty together with any other relevant information that might have emerged outside the MAP, and providing advice to the Minister regarding the affected PBS listing.

Patient consent and understanding

- Patients who are prescribed a medicine listed as part of a MAP must understand the process and provide informed consent. Specifically, patients must sign-off that they understand:
    - the risks involved in early access, including that the full risks and benefits of the medicine may still be under evaluation;
    - what data and evidence are being collected and why (eg. patients must understand what is being evaluated such as the clinical effect or the value of the drug); and
    - that the item may be 'de-listed' from the PBS pending the outcomes of the further data collection and assessment. Note that, in the circumstance of de-listing, the sponsor may be required to provide on-going access to individual patients who would benefit from continuing use of the drug.
- A communication strategy, targeted at patients and prescribers, that clearly articulates the arrangement is central to any new or continued MAP (including the possibility that a drug may not be reimbursed indefinitely).

- A systematic approach is required, that ensures transparency in the decision-making process and appropriate dissemination of information, such as through the National Prescribing Service.

Clinical trials

- In most cases where there are not sufficient high quality data to clarify the efficacy and safety of a medicine, randomisation in a Human Research Ethics Committee approved clinical trial is an appropriate standard of care. The cost of the new drug is not subsidised via the PBS in such trials. The MAP is not an alternative to conducting such trials in Australia.

Potential data issues

- The PBAC noted some potential issues with data that may be collected as part of a MAP:
    - entry of a new competitor to the PBS may confound data collected about patients receiving PBS subsidy within a MAP; and
    - timeframes for collection of data may be longer than anticipated.

4 Outcomes

4.1 The PBAC made specific amendments to the draft version (dated 11 March 2015) of the '*Framework for the Managed Access Programme for submissions to the PBAC'*, per Appendix A.

4.2 The PBAC would welcome the opportunity to provide further comment if required by the AMWG.