Vertex's response to Public Consultation on the draft revised Pharmaceutical Benefits Advisory Committee (PBAC) Guidelines

Executive Summary

In this submission, we set out Vertex's response to the Public Consultation on the draft revised Pharmaceutical Benefits Advisory Committee (PBAC) Guidelines.

The on-going review of PBAC guidelines is welcome, however, the current proposal represents a missed opportunity to develop a wider system that is fit for purpose for assessing all the medicines Australia patients need. A holistic system would include coverage of all rare medicines, this could include the Life Saving Drug Programme (LSDP) and existing Section 100 but also provide a tailored pathway for products that would otherwise fall in the "missing middle". This would allow a system that is:

- Tailored: This could incorporate a fast, flexible HTA review that leads to rapid access to innovative medicines for patients in need (as demonstrated in other countries):
- Focused: Special (PBAC) subcommittees should be created which would include subject matter/therapeutic area experts;
- Efficient: The process should be as fast as possible and not confined to PBAC's 17 week timetable; e.g. could meet in special session;
- Flexible: The process should incorporate Multi-criteria Decision Analysis (MCDA) or have appropriate exemptions.

Designing a mechanism that provides patient access, aligns prices to values, and provides sustainable funding for all medicines is challenging and an area of much debate. It is important to re-assess whether it is working effectively and meeting the objectives it was given. Vertex welcomes the opportunity to contribute to the debate on the Public Consultation on the draft revised Pharmaceutical Benefits Advisory Committee (PBAC) Guidelines.

A holistic system that is fit for purpose: appropriate for assessing al the medicines patients need

Given the focus on Vertex on developing medicines for CF, a rare disease, our primary comments focus on the extent to which the updated guidelines improve patient's access to orphan medicines. Subsidised access to orphan drugs in Australia has taken place through two main programs:

- The LSDP, aimed at rare medicines that are not cost effective, however, in practice there is some confusion as to whether the LSDP is intended for rare or very rare diseases. Since the LSDP was created, only 12 products have sought LSDP funding, 10 are currently reimbursed covering 7 disease areas. According to some authors, the LSDP has essentially narrowed its access to funding down to only very rare diseases.[i]
- Section 100 of the PBS i.e. the Highly Specialised Drugs Program[ii]. However, to be considered by the PBAC for inclusion in the programs, the treatment must demonstrate clinical advantages over existing treatments as well as cost effectiveness in comparison to these treatments.

Australia was innovative and early in its recognition of the special needs of medicines for rare diseases. In 1998, Australia introduced a special regulatory framework for orphan medicines designed to help manufacturers to overcome the risky nature of drug development associated with small patient populations, specifically a prevalence of 2,000 patients/subjects or less in the Australian population. In addition, the need for funding was recognised, hence the development of the LSDP. It was recognised that many products which carry an orphan drug designation by TGA are not cost effective if traditional rules of cost effectiveness are applied. In fact, the orphan drug category was created by the TGA in order to recognise the cost and risk involved in the development of these medicines is high and given the patient population these product would not otherwise be developed. This may be most extreme for medicines that could be categorised as "very rare" but nevertheless applies to all medicines for rare diseases.

This serves to highlight that there is a danger of a "missing middle" in the way medicines for rare diseases are subsidised in Australia. There is no approach for medicines with a larger patient population that are also inevitably going to be above standard cost effectiveness thresholds for medicines treating more common diseases – these are not addressed in the current draft guidelines. There are many medicines for rare diseases that are inevitably not cost effective by standard techniques given they serve relatively small patient population, and yet these are not eligible for a separate evaluation framework or subsidisation through a hypothecated fund such as LSDP. There is therefore a need for a third way – a tailored approach similar to Section 100 that allows for the unique characteristics of these medicines.

The DOH should consider introducing a new reimbursement pathway with a faster, more flexible HTA review, which would lead to rapid access to innovative medicines for patients in need. This could work by creating a new pathway for medicines for rare diseases that neither meet the requirement for LSDP nor meet the requirement for cost effectiveness set by section 100. Products in this channel could be exempted from parts of the HTA process such as in Germany (i.e. no comparator benefit assessment) or could still have a form of HTA but one based on MCDA as discussed above which would take into account the societal preference for access to different types of medicine for rare diseases. This new form of HTA could be led by special (PBAC) subcommittees which would include subject matter experts in specific therapeutic areas as well as patient representatives, called in for special sessions and therefore would not confined to PBAC's 17 week timetable. One example of this can be found in Scotland, where the Scottish Medicines Consortium convenes a Patient and Clinician Engagement (PACE) group which was introduced in May 2014 as a way for patients and clinicians to have a stronger voice in orphan (5 in 10,000) and ultra-orphan (1 in 50,000) medicine assessments. SMC considers "modifiers" in the HTA process that allows for higher uncertainty and cost per QALY.[iii]

This also illustrates the drawback of reviewing parts of the system in isolation. For example, the separate reviews undertaken to consider the Life Saving Drugs Programme (LSDP) in 2014 and the on-going review of the draft revised Pharmaceutical Benefits Advisory Committee (PBAC) Guidelines. In contrast, other countries are undertaking holistic reviews that focus on ensuring patients have rapid access to all innovative medicines delivering value to patients. For example, the Accelerated Access Review in the UK.[iv]

Conclusion

The current draft guidelines do not address the challenges facing medicines for rare disease. Medicines for rare diseases will inevitably not be judged as cost-effectiveness if applied against conventional criteria. This may be most extreme for medicines for "very rare" diseases but nevertheless applies to all medicines for rare diseases. Whilst a separate ring-fenced budget, such as LSDP, can make sense for some medicines (particularly those focused on very rare diseases), and other medicines for rare diseases may satisfy conditions for Section 100, there is a danger that other valuable medicines would not satisfy either regime – we describe this as the "missing middle".

The DOH/PBAC should re-consider following current international best practice and developing a new pathway to ensure all medicines for rare diseases are available to Australian patients. This means either providing exemption from parts of the HTA process to medicines for rare diseases or introducing a form of HTA that is based on multi-decision criteria analysis that involves both patients and rare disease experts. This more flexible approach along with a clearer definition of scheme eligibility, more specific binding guidance on the reimbursement process, and better communication throughout the process, would greatly speed up negotiations and improve access to innovative treatments for rare disease patients.

Endnotes

[i] "The Australian process for subsidised access to orphan drugs for rare inherited disorders of metabolism" April 2013, Vol 1, No 4 , Pages 273-277 (doi:10 1517/21678707 2013 772895) Read More: http://informahealthcare com/doi/abs/10 1517/21678707 2013 772895http://informahealthcare com/doi/abs/10 1517/21678707 2013 772895

[ii] Orphan medicines have always been funded by the LSDP, Section 100 and state level funding For example, in 2001, of the seventeen orphan medications that had marketing approval, seven had public funding Two were listed in the Section 100 of the PBS, one was supported under the lifesaving medication program and the final four were funded on a 50:50 basis by the Commonwealth and the States/Territories http://ses library usyd edu au/bitstream/2123/1008/2/02whole pdf

[iii] SMC (2014), "PACE (Patient & clinician engagement) overview document"

[iv] https://www gov uk/government/organisations/accelerated-access-review