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PBS Post Market Review Team
Department of Health
GPO Box 9848
Canberra ACT 2601

Dear PBS Post Market Review Team,

Thank you for the opportunity to comment on the items included in the PBS Guidelines Review. This review is a timely and important process that will ensure that Australia's Health Technology Assessment (HTA) process remains a robust and relevant part of the assessment of the value of new and innovative pharmaceutical products.

While the LSDP Review is ongoing, Shire believes that consideration should be provided within the Guidelines Review to policy decisions that may be made following the conclusion of the LSDP Review. Specifically, throughout the LSDP Review Shire has argued for a rare disease funding mechanism as follows:

- The creation of a standalone section under the PBS for the listing of medicines for rare and very rare disease (provided a working title in previous submissions of *Section 200*);
- Development of fit-for-purpose assessment criteria for products eligible for listing under *Section 200*; and
- The engagement of a committee with expertise in rare disease including representatives from Government, Industry, Clinicians and patients organisations to assist in the development of this fit-for-purpose assessment criteria.

The LSDP Review Reference Group acknowledged in their issues paper released in April, that there remains a need for a funding mechanism for medications for rare and very rare disease that are unlikely to meet the usual cost-effectiveness criteria applied within the Pharmaceutical Benefits Scheme. Understanding the difference between medicines for rare and very rare disease and those for more common conditions in fundamental to developing a funding structure in Australia that is fair, balanced and will meet the needs of patients living with a rare condition.

Should Government determine that funding for rare and very rare disease therapies should no longer be funded under the LSDP but rather, as part of the broader PBS then it is essential that this Guidelines Review take into account the unique challenges of developing treatments for rare and very rare disease including:

- Often a very limited pool of patients to generate clinical evidence in comparison with medicines used to treat more common conditions including:
  - The difficulty in determining clinically relevant endpoints for data collection;
  - The complexity of running clinical trial programmes and the difficulty in developing the typical evidence that is generally considered in a Health Technology Assessment;
- That these conditions can often involve significant individual variation in manifestation within a patient over time as well as between patients. These variations need to be taken into account not just at the initiation of therapy but also in making decisions about continuation of therapy; and

- Globally small cohorts of patients with rare diseases have a significant impact on the unit cost of medicines developed to treat these conditions;

Australia's current approach to HTA has an emphasis on meeting cost-effectiveness criteria. Given these challenges for rare and very rare disease, this approach is not sufficient to adequately assess the value of these products and an alternative mechanism is required.

To ensure that Australia's funding system adequately addresses the unique nature of products for rare and very rare disease Shire believes that a fit-for-purpose assessment process must be developed.

Issues that should be incorporated into these fit-for-purpose criteria include:

- An assessment of how the proposed treatment for a rare condition meet the unmet need in the target patient population;
- Severity and impact of the disease on the target patient population;
- Benefit provided by the proposed treatment to patients, carers and the community including not only benefit to life expectancy but also improvements in quality of life;
- The size of the patient population to be treated; and
- The overall budget impact of the proposed treatment
- Establishment of criteria for initiation and continuation of therapy.

Once again, thank you for the opportunity to comment. We look forward to the opportunity to provide further consultation and assistance to the review where required.

Yours sincerely

Brad Edwards
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Shire Pharmaceuticals