Guidelines Review Steering Committee
Pharmaceutical Benefits Advisory Committee Guidelines Review
Department of Health
GPO Box 9848
Canberra ACT 2601

11 September 2015

Re: Pharmaceutical Benefits Advisory Committee Guidelines Review

Thank you for the opportunity to comment on the items included in the Pharmaceutical Benefits Advisory Committee (PBAC) Guidelines Review. The most important outcome of such a review would be to ensure timely and sustainable access to new and innovative therapies that have a positive impact on the quality of life and life expectancy of Australians. It is therefore encouraging that the PBAC is seeking to develop more concise, clear, focused and up-to-date methods, and that the review process includes the assessment of methodologies currently used by comparable international authorities (items 1 and 2).

BioMarin is a global biopharmaceutical company committed to researching and developing treatments that address the serious unmet medical needs of patients living with rare diseases. As such, BioMarin has been involved in ongoing consultation regarding the Life Saving Drugs Program (LSDP) review, the findings of which are yet to be published.

BioMarin strongly believes in the need for a fit for purpose funding mechanism and assessment pathway for rare disease treatments. The current PBAC/LSDP assessment process for rare disease treatments is clunky and inefficient and therefore **the PBAC guidelines review needs to include as a separate item, the development of specific methodologies to assess and fund treatments for rare diseases** to ensure Australians living with rare diseases are not severely disadvantaged due to the unique challenges of assessing these products.

The unique challenges in assessing products for rare diseases include:

1) Cost-effectiveness
   The PBAC's current approach to Health Technology Assessment is weighted heavily on meeting cost-effectiveness criteria. Unfortunately, despite their important clinical benefits, treatments for rare diseases usually do not meet cost-effectiveness thresholds because of relatively high cost per patient (due to the high fixed cost of Research and Development and the need to recoup this investment from a very small population within limited market exclusivity periods). The cost-effectiveness approach therefore does not adequately assess the value of these products and an alternative mechanism is needed. Indeed the LSDP Post-Market Review Issues Paper (April 2015) acknowledged that "there remains a need for a funding mechanism for medications for rare conditions that are unlikely to meet the usual cost-effectiveness criteria applied within the Pharmaceutical Benefits Scheme". Alternative measures of value such as social willingness to pay[1] or Multi-Criteria Decision Analysis (MCDA) [2, 3] may be more useful when considering rare disease treatments.

2) Level of evidence
   Rarity means the volume and type of evidence may not be the same as in the case of conventional diseases.
   - Patient populations are small and heterogeneous.
   - There is often limited natural history data of the disease.

- There may be a lack of comparators and limited scientific understanding and consensus on appropriate clinically relevant endpoints.
- The time required to collect randomised controlled data for hard clinical outcomes such as survival (sometimes up to 10 years or more) may not be ethically defensible.

Therefore clear patient-relevant endpoints should be defined by considering the medical need and relevant criteria derived from literature review, past reimbursement decisions and consultation with patient groups, and should mirror the impact of disease for patients, families, society and payers.

3) Equity of access
Unlike a number of the major healthcare concerns facing Australia today, rare diseases are not lifestyle illnesses that patients have some control over. Australians living with rare diseases, many of whom are children, did not choose to be inflicted with a rare condition or the disability that often accompanies it. Australian human rights [4] and disability discrimination legislation [5] promotes the right to enjoy a life as normal and full as possible, the right to medical treatment, the right to measures that will enable individuals to become as self-reliant as possible, and the right to have their special needs taken into consideration at all stages of economic planning. These rights make it necessary to provide equity of access to treatments for rare diseases, as with common illnesses.

4) Social preferences for healthcare in Australia
Social preferences of the Australian community [1] should also be taken into account:
- a preference to prioritise health care for the worse off;
- a strong dislike for "all-or-nothing" resource allocation decisions;
- concern for the urgency of the initial health problem, especially if life-threatening;
- a preference for equitable outcomes over aggregate health benefits

These unique challenges of assessing rare disease therapies necessitate a rare diseases funding structure

Throughout the LSDP Review, BioMarin, together with other members of the Rare Disease Industry Working Group has argued for a rare disease funding structure that includes:

1) A separate section of the PBS for the listing rare and very rare disease therapies (eg *Section 200*)

2) A formal pre-submission process that determines eligibility for *Section 200* that may include:
   - Briefing document from the sponsor
   - Pre-submission stakeholder meeting including representatives from PBAC and the Department of Health, clinicians with specialty expertise in the disease, consumer representatives with knowledge of the disease, and the sponsor
   - Determination of the product's suitability for *Section 200* assessment
   - Consensus about possible initiation and continuation criteria for potential drug subsidation

3) A fit-for-purpose assessment process for products eligible for listing under *Section 200*, to be developed with the assistance of a committee with expertise in rare diseases including rare disease expert clinicians, government, rare disease consumer representatives and industry. The fit-for purpose assessment process would include:
   - Severity and impact of the disease on the target patient population
   - Assessment of how the rare disease therapy fulfils the unmet need in the target patient population
   - Benefit of the therapy to patients, carers and the community including not only impact on life expectancy, but also improvement in quality of life
   - The size of the patient population to be treated
   - The overall budget impact of the proposed treatment
   - Establishment of appropriate criteria for initiation and continuation of therapy

In summary, this review process is an opportunity to find a workable and sustainable program for patients, government and industry, and as such, **BioMarin recommends that an additional item be added to the PBAC guidelines review, to develop specific methodologies to assess treatments for rare diseases**.

BioMarin is committed to working in collaboration with other stakeholders to ensure Australians living with rare diseases have appropriate access to therapies in the future and looks forward to further opportunities to provide consultation and assistance to the review process.

Yours Sincerely

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References:

1. Richardson, J., et al., *Maximising health versus sharing: Measuring preferences for the allocation of the health budget.* Social Science & Medicine, 2012. **75**(8): p. 1351-1361.
2. Schlander, M., et al., *Incremental cost per quality-adjusted life year gained? The need for alternative methods to evaluate medical interventions for ultra-rare disorders.* Journal of Comparative Effectiveness Research, 2014. **3**(4): p. 399-422.
3. Sussex, J., et al., *A Pilot Study of Multicriteria Decision Analysis for Valuing Orphan Medicines.* Value in Health, 2013. **16**(8): p. 1163-1169.
4. *Australian Human Rights Commission Act*. 1986: Australian Government.
5. *Disability Discrimination Act*. 1992: Australian Government.