



Mucopolysaccharide &  
Related Diseases  
Society Aust. Ltd

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Dear PBAC Guidelines Review Panel,

### **RE: PBAC Guidelines Review Public Consultation**

The Mucopolysaccharide & Related Diseases Society Aust. Ltd is a national rare disease patient group representing Australian families living with Mucopolysaccharidosis (MPS) and related diseases. The Society welcomes the opportunity to provide input to the PBAC Guidelines Review Public Consultation. The Society would like to see the PBAC Guidelines strengthened via a more timely and efficient process and increased formal representation of the rare disease area including a rare disease health consumer, plus more involvement of patient groups.

**1. Review of the Guidelines content identifying the methods and current research. Identify significant new developments for methods in relevant sections of parts II and III of the current Guidelines.**

The Society calls for the Guidelines to be strengthened and incorporate specific guidelines that address the needs of rare diseases, in particular the limited data sets which are an inherent feature of rare disease. The Society recommends the PBAC Guidelines consider the nature of rare disease and how to appropriately manage risk and small data sets from across local and international sources.

**2. Preparation of a technical paper for discussion on each issue for the revision, including identifying any issues of scientific debate and consideration of Australian and International best practice.**

The Society agrees with the dual consideration of local and International best practice. This is particularly pertinent for rare diseases which Australian scientific knowledge may be limited.

**3. Consolidation of these into a revised set of Guidelines for methods (with any matters related to process identified and agreed as out-of-scope)**

The Society calls for the PBAC to adopt a formal process for listing rare disease treatments in a more efficient and timely way, rather than the current system which may see rare disease treatments listed only once they go through and 'fail' general PBS criteria. The Society would also favour a formalised process that includes a PBAC directed stakeholder meeting to consider the listing of a rare disease treatment, including representation from the patient group. The Society has recently been involved in a sponsor-led stakeholder meeting which included equal and effective representation from Department of Health, expert clinicians and the Society. This was a positive experience and we believe should be a formal feature of the PBAC process in regards to rare disease treatments.

**4. Management of a public consultation process for the proposed revised Guidelines for methods.**

The Society supports a public consultation process for the proposed revised Guidelines for methods.

**5. Incorporate accepted consultation feedback into the draft revised Guidelines**

The Society recommends that rare diseases are considered differently to common conditions as experiencing the following inherent challenge: 1 diagnosis delay and misdiagnosis are common; 2.lack of information; 3 lack of scientific knowledge; 4. Lack of treatment options and high cost of few existing treatments; and 5. Inequities in access to treatment.

**6. Present the finalised revised Guidelines for consideration by the DUSC and the Economics Sub Committee ESC, and for endorsement by the PBAC.**

The Society would also like to see the discussion paper with draft revised Guidelines made available to for public review.

Yours sincerely,

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