

Public Consultation on the Post-market Review of PAH

Submission by Dr Richard Whitaker.

I am a medical practitioner and also the father of a child who died from PH. I work with Pulmonary Hypertension Australia, the major support group for patients with PH.

The draft Review Terms of Reference are:

- 1. Review recent clinical guidelines for the management of PAH and compare this to the PBS restrictions and Therapeutic Goods Administration (TGA) indications for the use of PAH medicines.**

It is difficult for health practitioners, and impossible for patients, to understand why patients in Australia are provided different treatments according to the aetiology of their PH. This is not the case overseas and patients are well aware of this.

Many patients would gladly participate in clinical trials, but overall numbers are so low that any meaningful data arising from these trials is unlikely.

I, and many patients, would like to see the Australian clinical guidelines for the management of PAH reflect overseas experience and align with international guidelines.

- 2. Review the utilisation of PAH medicines in Australia, including sources of data that can provide additional information on clinical use that is not available from PBS data.**

Patient support groups will provide some evidence for the clinical use of PAH therapies. There is still a significant treatment cost borne by patients and their families. Many regional hospitals support patients with PAH in conjunction with the specialist PAH centres. Significant expenses are incurred by these regional hospitals in providing medication and other therapies for PAH patients.

- 3. Review the clinical outcomes that are most important or clinically relevant to patients with PAH, and the extent to which these outcomes are included in the evidence previously considered by PBAC.**

Traditional outcome measures such as mortality are no longer suitable for measuring the impact of PAH on patients. The increasing utilisation of PAH medications, including combination therapy, has meant that many patients are living longer and quality of life (especially considered in terms of activities of daily living) is a much more relevant measure of outcome. Patients are likely to remain in meaningful employment for long periods of time, are still being married and raising children, and remain active in community life. The severity

of symptoms is a major limiter for many patients, and this is a very meaningful outcome measure for many patients.

4. Collate and evaluate evidence on the comparative effectiveness of PAH medicines, including combination use and use in the WHO functional class II patient populations.

Again, it is difficult for health practitioners and patients alike to understand why more combination therapy is not available in Australia. Australian patients are very well informed and interact through social media with patients and patient groups overseas. There is a significant disparity in treatment options that patients are very well aware of.

Patients have significant issues with the absolute six-minute walk test (6MWD). Despite there being a national guideline on how to perform this test, patients report significant disparity between its performance in different centres, and also at different times in the same centre. Patients from regional centres are often compromised by long travel and frequently feel they have not performed their best. There is also evidence that this test is not as good a marker of disease treatment or progression as once thought. Again, different outcome measures are called for in the management of PAH in Australia.

5. Following TOR 1-4 consider reviewing the cost-effectiveness of existing PBS listings for PAH medicines, and in treatment of WHO functional class II and combination treatment in class III and class IV patients.

From a patient viewpoint I believe that the cost-effectiveness of these medications needs to be assessed with regard to new patient outcome measures. Again, I think that mortality should be a less prominent outcome measure and the patients' ability to continue to live a 'meaningful' and 'productive' life should be substituted. Some of these outcomes are clearly qualitative, but some will be quantitative (many PH patients on treatment are contributing to the Australian economy).

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