phaa
PULMONARY HYPERTENSION ASSOCIATION AUSTRALIA

PHA AUSTRALIA INC.
ABN: 86 815 330 924

TOGETHER WE ARE STRONGER

TO THE PAH REVIEW SECRETARIAT:

PHA Australia is registered non-profit organisation, managed by an elected committee. Our main focus is to provide support, education, create awareness and provide the latest information and research on Pulmonary Hypertension. We have several thousand members across all platforms made up of patients, carers and family members.

Draft Review Terms of Reference -

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.**

   Clinical guidelines for management of PAH are above most patients understanding'. However, our members find it difficult to understand why in Australia we don't have the same access to the range of drugs or the ability to have multiple PH drugs at an affordable cost. Members rarely hear about clinical trials and would certainly take part if given the opportunity.

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.**

   A large proportion of our members are on combination treatment for their PAH, exact figures are unavailable due to privacy constraints. Due to the PBS restriction of only one medicine patients rely on hospital funding of drugs, compassionate supply by the drug companies or self-funding to enable use of additional treatments. Only those that are financially able to can self-fund, many selling property or cashing in retirement savings to do so. These drugs are extremely expensive and the costs involved cause stress and anxiety among patients as they fear drugs being cut at any time.

   We have many members across Australia treated in large centres of excellence in major cities and others in smaller centres in cities regional areas. Because PAH is a rare disease it is critical that all patients, regardless of where they live, are offered the best possible care and to achieve this there needs to be a greater emphasis on linkage between expert centres who see high numbers of patients and have very experienced doctors and the non-expert centres that see a small number of patients.

The recent change to the requirements for continuation of PBS supply of PAH medicines has been viewed as positive by our members. Members have always been anxious and stressed each time they go for their 6 monthly review and the critical emphasis placed on the 6 minute walk test result to ensure they continued medication.

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.**

Most studies concentrated on assessing changes in exercise capacity. It is true that our members value that exercise but equally they value how they feel, how they function day to day, and whether their treatment is going to keep them out of hospital and alive. Some members are acutely aware that if they end up in hospitals it can signal that it sadly could be the start of the end of their time. Because it is a rare disease limited patient numbers are available in Australia and so it can be hard to conduct clinical trials here. Regional patients are essentially excluded due to the distances required to travel regularly to the trial centre in major cities. Financial burden on patients and families is a very large factor.

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.**

It seems senseless that PAH medicines are only reimbursed for PAH that has come about for particular reasons. An example is PAH due to drugs and toxins, which to our knowledge, has no access to reimbursed treatment in Australia even though they are a relatively small group of patients. If a patient is lucky enough to be diagnosed as functional class II (very few) it doesn't make sense to have to wait until the disease worsens to class III before you have access to reimbursed medicines. PAH is PAH regardless of functional class and a small number of patients are discriminated against on the basis of how they got their disease and or how sever it is.

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.**

The cost-effectiveness of these medications needs to be assessed with regard to new patient outcome measures. Mortality should be a lesser outcome measure and the patients' ability to continue to live a productive life with quality should be substituted. There also needs to be recognition of the disease in all government but more importantly in Centrelink. Members are frustrated that they need to educate consultants every time they go to apply for any benefits.

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