12 May 2016,

To the Chair of the PBAC

RE: PAH Review Draft Terms of Reference

To whom it may concern,

Pulmonary Hypertension Society of Australia and New Zealand (PHSANZ) Submission on Terms of Reference (TOR) for the Pharmaceutical Benefit Scheme (PBS) Post-market Review on Pulmonary Arterial Hypertension (PAH) medicines:

The PHSANZ is a not for profit scientific society representing over 300 medical practitioners/ Nurses/allied health professional members within the field of Pulmonary Hypertension across the region of Australia and New Zealand. Our mandate is to advocate for sustainable models of care that promote timely and equitable access to skilled clinical staff and timely and appropriate access to lifesaving medicines for patients with pulmonary hypertension. The aims of the PHSANZ include addressing issues in the area of pulmonary hypertension that are the core concerns of patients and their clinicians. Our registry aims to collect data to allow review of demographics, management of PAH and audit of the outcomes across Australia to further improve patient outcomes. Currently, it contains clinical and outcome data for over 3000 Australian and NZ patients with pulmonary hypertension

The PHSANZ is delighted that this therapeutic area is under review as we believe that current funding and prescribing criteria for PBS funded therapy are no longer in line with current international guidelines and nor do they reflect the current approach to treatment in Australia[(1)].

The current terms of reference are indeed important areas for consideration and we applaud the PBS for the initiating this review. The current TOR 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.
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.
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.

Pulmonary Hypertension Society of Australia and New Zealand Inc.
PO BOX 133, Sans Souci, NSW
ABN 11407595406

4. Collate and evaluate evidence on the comparative effectiveness of PAH medicines, including combination use and use in the WHO functional class ll patient populations.
5. Following TOR 1-4 consider reviewing the cost-effectiveness of existing PBS listings for PAH medicines, and in treatment of WHO functional class ll and combination treatment in class lll and class lV patients

In regard to the above TOR and in addition, the PHSANZ would suggest the following be key areas of focus to be explored fully by the review of PAH Medicines.

a) Under (1) & (4) above we would emphasise the key concerns of Australian patients and clinicians specialising in this field: (i) that early access to therapy (for patients in Functional Class II) be available and (ii) that early use of combination therapy be available if patients remain in a higher risk category. These aspects, are now high priorities, being evidence based and recommended by international guidelines.

b) Again under (1) the TOR should include the review of the criteria for starting therapy and specifically, the need for a several week trial of vasodilator therapy. The PHSANZ would remind the committee of our continued concern with respect to the PBS mandated trial of a calcium channel blocker for eight weeks (if mean right atrial pressure <8mmHg) across all WHO Group 1 patients. This is not supported by evidence and that this blanket requirement should be removed [(2)]. *It can even be dangerous, via adverse effects on the impaired right ventricle.*

c) Under (2) the TOR should include comparison of available access to funded therapy for Australian patients to the utilisation of such therapies in comparable public funded health systems in OECD countries in Europe and North America.

d) Under (1) and (2) The equitable access to lifesaving medicines is not feasible in many centres, due to a lack of support staff and funding for equipment to manage IV therapies for PAH. Utilisation of iv epoprostenol per capita in Australia is significantly lower than published figures for other OECD countries. Lack of the above mentioned resources limits the equitable access to services for PAH patients in those centres that do not have these additional support services. Under (2) and (3),) treatment by experienced clinicians with a specialist interest in pulmonary hypertension has been shown to improve outcomes and ensure appropriate use of therapies as demonstrated in UK data stretching over 10 years [(3)]. The PAH review TOR should include a review of current practice which has led to 58 centres in Australia (UK has 7 and France 26 for much larger populations), a situation which, even allowing for the geographical challenges, is difficult to defend. However, decisions on escalation of therapy require significant expertise and we would strongly support the TOR including a review of the most effective method of ensuring prescribing is in the hands of experienced clinicians. The overseas data also demonstrate that use of specialised centres has avoided overuse of medication by improving adherence to guidelines.

e) Further, related to designation of centres, the TOR should review what constitutes a designated Pulmonary Hypertension centre. Access to lifesaving medicines is inequitable, being unavailable in some centres due to a lack of support staff to manage intravenous therapies for PAH. Utilisation of iv epoprostenol per capita in Australia is significantly lower than published figures for other OECD countries. This is indeed,

limiting the equitable access to services for PAH in those centres that do not have these additional support services. This may include review of existing designated centre status and revision of minimum requirements for prescribing centres, taking into account published international guidance on this matter but also considering Australian geographical constraints. To consider longitudinal audit of existing centres to ensure maintenance of defined prescribing centre standards and participation in quality assurance activities, such as the national registry.

f) Whilst we support the removal of the continuation rules for prescribing PAH medicines, The PAH review should review the implications of removing the discontinuation rules without ensuring patients on therapy are monitored appropriately with 6 monthly echocardiogram and six minute walk test and should evaluate the consider the value of mandating regular review by prescribing physician. This would contribute to management guidance and how to enable the PBS to maintain long term oversight of treatment utilisation and patient outcomes either directly by feedback at time of extension of funding authority or by using the PHSANZ Registry data.

The PHSANZ would like to again offer its services as an expert resource for the PAH review and would be very willing to collaborate with this Review. Therefore, we would encourage PBS to consider utilisation of the data in the PHSANZ registry to inform their discussions and decisions. These data are collected with the purpose of improving patient care and the Society is open to requests for specific analyses by a wide range of stakeholders in this area.

Your sincerely,

[redacted]

A/Prof Ken Whyte
PHSANZ President

Prof Eugene Kotlyar
PHSANZ President Elect

cc: PHSANZ Board

References

1. Galie N, Humbert M, Vachiery JL, Gibbs S, Lang I, Torbicki A, et al. 2015 ESC/ERS Guidelines for the diagnosis and treatment of pulmonary hypertension: The Joint Task Force for the Diagnosis and Treatment of Pulmonary Hypertension of the European Society of Cardiology (ESC) and the European Respiratory Society (ERS): Endorsed by: Association for European Paediatric and Congenital Cardiology (AEPC), International Society for Heart and Lung Transplantation (ISHLT). European heart journal. 2016;37(1):67-119.
2. Sitbon O, Humbert M, Jais X, Ioos V, Hamid AM, Provencher S, et al. Long-term response to calcium channel blockers in idiopathic pulmonary arterial hypertension. Circulation. 2005;111(23):3105-11.
3. Corris PA. The UK National Pulmonary Hypertension Service, Registry and Research Collaboration. Glob Cardiol Sci Pract. 2015;2015(3):37.