rare voices
AUSTRALIA

3rd April 2016

Dear PBAC Guidelines Review Secretariat,

Re: PBAC Guidelines Review Public Consultation

Rare Voices Australia Ltd. (RVA) welcomes the opportunity to respond and provide input to the PBAC Guidelines Review Public Consultation. RVA is keen to see the PBAC Guidelines strengthened by creating efficiencies in process, representation for the rare disease sector from a range of stakeholders including the rare disease health consumer and consider the rare disease principles and objectives to enable safe and effective treatments to be readily accessible to the rare disease community. People living with a rare disease live in the hope of one day a treatment will be discovered and that the process is a fair, equitable and transparent one with efficiencies in place to ensure treatment listing is efficient and without delay.

If the PBS will include items currently listed on the Life Saving Drugs Program (which is currently undergoing a review) RVA recommends that the PBAC Guidelines consider the nature of rare disease and how to best manage risk as well as small data sets from across local and international sources. A 'fit for purpose model' needs to be considered as part of the scope of listing rare disease treatments under the PBS.

RVA proposes that the PBAC adopt a formal process for listing rare disease treatments that considers the need to allow efficiency in process avoiding lengthy time delays. A process whereby PBAC directs a stakeholder meeting to consider the listing of a new rare disease treatment with equal representation from a range of stakeholders; rare disease clinical / research experts, PBAC Director/ representative, TGA representative, Sponsor company representative, Rare Disease patient organisation / RVA representative. To date this has been trialled by former PBAC Director and recently by Department of Health under the sponsor company direction. The process by which this occurs should be consistent across all rare diseases and the facilitation should be from PBAC with agenda, minutes and outcomes made transparent to all involved.

The stakeholder meeting allows all relevant materials to be reviewed, clarification of data and questions raised and answered in a collaborative environment. Key outcome to bring all necessary data to PBAC to consider and make its decision based on information from local and international data. As part of best practice, the meetings need to state objectives, outcomes and minute discussions. All to be made available for public review.

This process will enable and address the time delays for resubmitting to PBAC if a decision is deferred or rejected. Whereby additional data is required, the process and manner in which this is addressed again needs to take into consideration the burden that process has not only on the Sponsor Company but the patients themselves. The key outcome is to improve inefficiencies in a system that currently does not formally consider rare disease treatments. Thus looking at how to improve process which ultimately improves health outcomes for health consumers in Australia.

RVA recommends that rare diseases are considered in a different light to that of common conditions. Some important points to consider that are shared amongst the many variety of rare diseases include;

I. Lack of access to correct diagnosis

The specific and challenging characteristics of RD result in patients experiencing late or inaccurate diagnosis, fragmented access to care and inadequate or inappropriate treatment proving both harmful and of great risk to the RD patient.

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II. Lack of information
Dissemination of scarce existing information is a key issue for the rare disease community at large.

III. Lack of scientific knowledge
This results in difficulties in the areas of therapeutic tools development, definition of the therapeutic strategy and in shortage of therapeutic products, both medicinal products and appropriate medical devices

IV. Social consequences
Living with a RD has implications on all areas of life; school, work choice, leisure time are all affected leading to isolation, exclusion from social community, discrimination for insurance (health, travel and mortgage insurance) and reduced professional opportunities.

V. Lack of appropriate quality healthcare
Appropriate healthcare would entail combining the different spheres of expertise needed for rare disease patients and adopting appropriate models of care. The “Multidisciplinary” clinic models have been adopted for some of the rare diseases in Australia and have resulted in treatments being listed on either the Life Saving Drugs Program or PBS (Pharmaceutical Benefits Scheme).

VI. High cost of the few existing drugs and care
The additional expense of coping with the disease (human, medical and technical aids), combined with the lack of social benefits and reimbursement, always leads to an overall pauperisation of the family, and dramatically increases the inequity of access to care for rare disease patients. Most often families simply cannot afford the best available healthcare for their loved ones.

VII. Inequities in availability of treatment and care
Innovative treatments are unevenly available across the world including Australia. Clear contributing factors include; economy, scarce priority given to rare diseases within the Public Health sphere. Additionally delays such as; price determination and/or reimbursement decision, lack of experience of the treating physicians (not enough physicians involved in rare diseases clinical trials), and the absence of treatment consensus recommendations in Australia and globally is an ongoing problem.

Rare Disease Treatments need to be Accessible via a seamless and efficient process
Rare Disease treatments need to be accessible. The process by which they are listed and reviewed needs to recognise the people it is set up to serve being the people who are impacted each day living with a chronic, life threatening, debilitating condition whereby no other treatment option is available to them and the treatment will potentially bring improvement to their quality of life.
According to the report; ‘Funding Rare Disease Therapies in Australia Ensuring Equitable Access to healthcare for all Australians’ (The McKell Institute), there are inefficiencies experienced in Australia compared to that of international comparable countries with regard to accessing therapies for rare diseases. (1) The average orphan drug marketing application is taking on average 15 months to be approved including the pre-submission process. In comparison to the United States and Europe the orphan drug marketing applications are evaluated in 10 months or less.
There is opportunity for the PBAC to further streamline process and speed access to innovative orphan drugs for rare diseases.

Principles
RVA recommends that some shared Principles that underline rare disease be adopted and linked to the Orphan Drug Program for Rare disease and considered as part of the review of the PBAC Guidelines.

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1. Social Justice, Equity and Solidarity principles

Based on the principles of social justice, equity and solidarity, the needs of rare disease populations have to be specifically addressed, as they should be for any minority or underserved community. This is an important part of the goal of reducing health inequalities for rare diseases in Australia.
People living with a RD are a major healthcare concern and unlike other key health priorities they are not the result of 'poor lifestyle choice i.e. smoking, drinking.' Australians living with a RD are born with their RD and the disability that also is associated with it.

Conclusion

Rare Voices Australia would like to acknowledge the PBAC, Department of Health and the Life Saving Drugs Program Reference Committee and other department's willingness to engage with RVA allowing the patient community voice to be included and heard in the *PBAC Guidelines Review Public Consultation*.

Australian patients living with rare diseases call for the Australian Government to adopt a National Rare Disease Strategy and it is pleasing to report that the Western Australian Department of Health have now published a Western Australian Rare Diseases Framework 2015-2018 endorsed by WA Director General of Health. Rare Disease patients need to access treatments that have been approved by the TGA as safe and effective in a system that adheres to a transparent, efficient and equitable process. They need to have confidence in the PBAC who understand rare diseases and importance of access to safe effective therapies and treatments. Most of these medicines and therapies are innovative, new and some are complimentary or in addition to existing medicines or products. Some rare disease treatments are specifically targeted towards very rare diseases. There needs to be flexibility in the approach to listing such treatments and guidelines drafted need to consider this carefully to ensure patients who can benefit from accessing them, will in fact receive them in a timely manner to optimise opportunities to show and receive clinical long term benefit.

Kindest Regards,

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