rare voices
AUSTRALIA

Life Saving Drugs Program Post Market-Review
Pharmaceutical Evaluation Branch, Pharmaceutical Benefits Division, Department of Health
GPO Box 9848, Canberra ACT 2601

10 November 2014

Dear Life Saving Drug Program Post-market Review Independent Reference Group

Re: Public consultation on the Post-market Review of the Life Saving Drugs Program (LSDP)

Rare Voices Australia Ltd. (RVA) welcomes a review of the Life Saving Drugs Program and the opportunity to submit its recommendations for consideration as part of the Review.

Rare Voices Australia Position Statement
"RVA would like to see a program that allows patients living with rare life limiting diseases the ability to access safe, effective, appropriate treatments that potentially bring a positive impact to the quality of life and life expectancy of Australians living with rare diseases."

Careful consideration needs to be given to the National Rare Diseases Plan that has been recommended for adoption by the rare disease community; patients, clinicians, industry, policy makers at the Inaugural International Symposium on Rare Diseases 'Awakening Australia to Rare Diseases' held in Fremantle, Western Australia in April 2011. The symposium was the impetus for Rare Voices Australia forming. (1)

Rare Voices Australia Background
Rare Voices Australia, the National Alliance of the rare disease community is a unified voice of 1.2 million Australians living with a rare disease. RVA members compromises of over 200 rare disease patient organisations throughout Australia including adults and children living with a rare disease. RVA's Mission; the national alliance of people living with a rare disease, will provide a unified voice to improve the lives of all Australians affected by rare diseases.

Rare Voices Australia Vision: Australians with rare diseases will have extended and improved lives. Access to world class, improved, better, best practice in areas of; diagnosis, treatment, care, services, support services and therapies. Reducing mortality and morbidity enabling the 'best' life possible.

What is a Rare Disease?
A rare disease is a disease that occurs infrequently or rarely in the general population.
Recently the Australian rare disease community has proposed a definition of rare disease (RD) as being;
'a life-threatening or chronically debilitating disease which is statistically rare, (with an estimated prevalence of less than 5 in 10,000 or of similarly low prevalence) and has a high level of complexity such that special combined efforts are needed to address the disorder or condition.' (2) This definition is similar to that promulgated by the European Commission on Public Health. This is the definition Rare Voices Australia has adopted.

Rare Disease shared features and experiences
RD's are diverse collectively RD's share common features;
1. 80% of RD are genetic.
2. Approximately 5000 and 7000 distinct RD exist today.
3. Great diversity in the age at which the first symptoms occur varying from new born to 50 years.
4. Symptoms may be evident in childhood but may not translate into a specific rare diagnosis for years. Symptoms of many RD's appear at birth or in children or may not become apparent until adulthood.
5. Rare diseases are usually severe to very severe and chronic
6. Often degenerative and can be Life threatening
7. Disabling; the quality of life is often compromised by lack / progressive loss of autonomy
8. Psychosocial burden: the suffering of RD patients and families is aggravated by isolation, the lack of therapeutic hope and the absence of practical support for everyday life.
9. RD are difficult to manage; patients encounter difficulty locating adequate treatment, support and social services.
10. RD don't have a cure. In some cases symptoms can be treated to improve the quality of life and life expectancy.

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Rare Disease Shared Experiences;
Despite the diversity of RD's and the wide variety of symptoms, RD patients and their families are confronted with the same difficulties and everyday challenges. These include;

1. 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.

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

3. 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.

4. 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.

5. 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 the LSDP.

6. 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.

7. 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.

What are the agreed key principles for a rare disease treatment program?
RVA supports a program that operates on agreed, shared principles and works in conjunction with a National Rare Diseases strategy yet to be formally adopted in Australia.

The following Principles need deliberation in an open Rare Disease Forum with all key rare disease stakeholders (patient organisations, clinician, and pharmaceutical industry, policy makers) to share all perspectives of the various stakeholders to form consensus.

Principles may include:
1. Patients living with rare disease need a National Rare Disease Strategy
Australian rare disease patients need a National Rare Disease Plan. (3)

- Diagnosis – access to appropriate, cost-effective testing
- Access to current, relevant and appropriate information for patients and health professionals
- Access to support services
- Rare disease research development
- Rare Disease data, national registry, collection of data
- Clinical management of the rare diseases

2. Evidence / Data is limited in Rare Diseases

Access to rare disease data in our country is often difficult and limited.

- Rare diseases are very different to non-rare disease and the level of data available to show robust evidence typically seen in non-rare disease treatments is limited.
- Patient populations are typically small and heterogeneous therefore there is very limited natural history data of the disease.
- Often there is very limited scientific understanding and consensus amongst local practicing clinicians.

3. Access to treatments needs to be an equitable, efficient and transparent process

Improving access to safe, effective and appropriate treatments, medicines and medical devices for rare diseases is crucial.

- The Australian rare disease community need to have a program that adopts clear values and guidelines that ensure a transparent process in which all rare disease stakeholders (patients, clinicians, industry) understand and respect.
- A program that promotes global best practice delivering safe, effective and appropriate treatments in a timely, efficient manner.
- The program should be transparent, fair, equitable mindful of the end user being the rare disease patient.
- The patient community need to be a part of the process from planning to delivery.

4. **Cost-Effectiveness**

Timely access to medicines that Australians need at a cost that the individuals and the community can afford is essential.

- The program for administering RD treatments needs to adopt efficiency in access.
- Negotiation on cost of treatments is too long and is inefficient process. The process lacks transparency.
- Patients are 'stuck in the middle', medically compromised and desperately campaigning or 'begging for lives'. This scenario is not only unethical but long term unsustainable. We have a system that has reduced to whoever screams the loudest gets what they want.
- Provide incentives for RD Researchers to set up sites to enrol patients into clinical trials to further investigate RD's and the benefits of accessing particular treatments.

5. Principles of Social Justice, Equity and Solidarity

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.

The UN Declaration of Human Rights (Article 25.1) states:
"Everyone has the right to a standard of living adequate for the health and well-being of himself and of his family, including (…) medical care and necessary social services, and the right to security in the event of (…) sickness, disability, or other lack of livelihood in circumstances beyond his control."

The International Covenant of Economic, Social and Cultural Rights (Article 12.1) includes "the right of everyone to the highest attainable standard of physical and mental health" (Article 12.1). Article 12.2 of this covenant further provides: "to realise these rights, steps must be taken by the state to reduce infant mortality, to improve hygiene, to prevent, treat and control diseases and assure all medical service and medical attention in the event of sickness."

The UN Convention on the Rights of the Child (1989) includes right to the highest attainable standard of health care.

The Australian Human Rights Act (4) and disability discrimination legislation (5) promotes the right to enjoy a life as normal and full as possible, the right to medical treatment, the right to measures that will enable individuals to become as self-reliant as possible, and the right to have their special needs taken into consideration at all stages of economic planning. While it is acceptable that the implementation of these instruments is subject to the laws, policies and budgets of its jurisdiction, it is not acceptable that public health planning and services often give so little priority to the needs of rare diseases patients. The consequence of this lack of interest has been to reinforce the disadvantages of people living with rare conditions in Australia.

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A **Response to Terms of Reference**

1. Review the clinical effectiveness and safety of medicines currently subsidised through the LSDP

RVA believes this is the role of the TGA who oversees the clinical effectiveness and safety of medicines. The LSDP, PBAC, PBS need to collaborate with the TGA.

2. Review emerging clinical treatments and diseases, including those that identify sub-groups by molecular target, which could potentially seek subsidisation through the LSDP in the future

RVA doesn't have access to this comprehensive data. EURORDIS (The European Organisation of Rare Diseases) and NORD (The National Organisation of Rare Disorders –United States of America) receive Government funding to prepare this data on a regular basis. TGA does have a listing of treatments that have received approval locally. It could be adopted to include further information.

3. Conduct an international comparison of subsidisation of drugs for rare diseases and the definitions for a rare /ultra-rare disease

Internationally there is no differentiation of rare and ultra-rare disease. RVA has attended to the definitions of what is a rare disease within the context of this submission.

4. Compare the subsidisation and equity principles of the PBS and the LSDP.

The PBS is designed to list treatments that have shown to be safe, effective and affordable treatments. Are they appropriate? This is in the hands of clinicians and patients rely on them to guide them to the best advice.
How does the average GP manage a RD patient whom may have very little knowledge about the disease?
What is clinical best practice? How is this best managed?
The LSDP principles looked at how to manage treatments that are safe, effective and appropriate but they don't meet cost-effectiveness of the PBS model and there are no other alternative therapies for that particular disease. In principle the LSDP from a distance appears to work. But in reality is it delivering?

The treatment programmes coordinated by the LSDP are not always lifesaving, but can play a major role in limiting disease progression and maximising the RD patients' ability to contribute fully to the wider and immediate society. To that end the eligibility guidelines in place for the 10 listed treatments for the 7 rare diseases are arbitrary and in some cases discriminatory.
RVA acknowledges that the Australian Disability Discrimination Act 1992 promotes recognition and acceptance within the community of the principle that persons with disabilities have the same fundamental rights as the rest of the community.(5) The Australian Human Rights Commission Act 1986 promotes the principle that every person is equal in dignity and rights (4). People who have a disability (including people living with a rare disease), have the same fundamental rights as their fellow citizen of the same age. This implies the right to enjoy life to their fullest potential. People living with a RD are entitled to the measures designed to enable them to become self-reliant as possible and they have the right to medical treatments which will enable them to develop and reach their full capacity. The Act states that disabled persons are entitled to have their special needs taken into consideration at all stages of their economic and social planning.
Withholding treatment from Australians who are diagnosed with a rare disease whereby there is a safe treatment that has shown clinical benefit to them, is going against the person's fundamental human right. Patients living with a rare disease simply cannot afford the high costs associated with accessing a treatment.

5. Assess the value for money of the medicines subsidised on the LSDP by evaluations the benefit of each drug's treatment outcomes, including in terms of quality of life achieved through the program and their cost.

How does the LSDP Review Committee define the term 'Value for money'? What is the value to a person's life? How can this be best measured?
In the context of a patient living with a rare disease, who in most cases been born with their disorder, the disorder is not that of lifestyle choice, overeating, smoking or drinking. People who are diagnosed with a rare disease have the right to health and they have the right to access best possible care and treatment.
RVA recommends that an appropriate definition of value for money could include the value of improvement of quality of life and life enhancing benefits as well as the costs and impacts on patients not on treatment who progressively get worse and therefore are a 'burden to the public health system'. Research has suggested that medicines for RD may be best valued using mulit-criteria decision analysis that includes all relevant stakeholders, including patient groups (6).According to the Health Technology Assessment World Europe, it is anticipated that budget impact of RD treatments will plateau in 2016 at 4-5% of total drug spend (7)

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6. Review the administration of the LSDP, including the Guidelines with which the program is administered for each condition, and assess alternative administrative systems.

The guidelines for receiving funded therapy need to be reviewed on a regular basis in an efficient manner. The process by which they are reviewed has the potential to improve in round table stakeholder meetings. RVA has consulted with the patient organisations that have treatments listed on the current program and some would like to see the guidelines reviewed to reflect those of the UK and Europe. RVA would like to see that international guidelines used to review and revised with the current guidelines in Australia There is a joint declaration for rare diseases on international public health priority drafted with ten recommendations. RVA sits on the IRD (International Rare Diseases) and would like to see Australia collaborate with international rare disease organisations and review the current administrative system. The Rare Disease Forum of RD stakeholders could further deliberate on this point in early 2015 to bring its consensus view to the review panel with its recommendations.

7. Establish a framework for data collection on rare diseases in Australia and assess how this could function internationally.

There is a need to develop Internet-based rare disease registries to support health care stakeholders to deliver improved quality patient outcomes. Such systems should be architected to enable multiple-level access by a range of user groups within a region or across regional/country borders in a secure and private way. However, this functionality is currently not available in many existing systems. A new approach to the design of an Internet-based architecture for disease registries has been developed for patients with clinical and genetic data in geographical disparate locations. The system addresses issues of multiple-level access by key stakeholders, security and privacy. The system has been successfully adopted for specific rare diseases in Australia and is open source. The results of this work demonstrate that it is feasible to design an open source Internet-based disease registry system in a scalable and customizable fashion and designed to facilitate interoperability with other systems. (8) Rare Voices Australia in collaboration with Murdoch University have received a Wellcome Trust Award to develop an Independent Rare Disease Registry. The pilot of IRDR will be delivered by end of 2015.

Conclusion

Rare Voices Australia would like to acknowledge the Department of Health's (Dept) willingness to engage with RVA allowing the patient community voice to be included and heard in the LSDP Review. RVA held an independently facilitated meeting in Melbourne on 4th September inviting members of the Dept to talk with rare disease patient organisation leaders affected by the LSDP review in a round table forum. The forum allowed transparent conversation about the LSDP review and the submission process. The process proved beneficial to all who attended and RVA received a very positive response from the patient organisation groups. A written report has been prepared and circulated to the attendees including the Dept as a record.

Australian patients living with rare diseases call for the Australian Government to adopt a National Rare Disease Strategy. Rare Disease patients need to access treatments that have been approved as safe and effective in a system that adheres to a transparent, efficient and equitable process.

It is a basic human right for rare disease patients to expect access to treatments within their home country.

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