



rare voices

A U S T R A L I A
14th July 2015

Committee Secretary
Senate Standing Committee on Community Affairs
PO Box 6100
Parliament House
Canberra ACT 2600

Submission to the Senate Community Affairs Legislation Committee Inquiry into the Medical Research Future Fund Bill 2015 and the Medical Research Future Fund (Consequential Amendments) Bill 2015.

Rare Voices Australia is the National Alliance of the rare disease community and representative of an estimated 1.2 million Australians living with a rare disease. RVA members comprises of over 200 rare disease patient organisations throughout Australia including adults and children living with a rare disease. RVA advocates for a national approach to rare disease including access to rare disease health and medical research in Australia.

Rare Voices Australia's Mission / Vision is for Australians living 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.

RVA believes that the Medical Research Future Fund Bill 2015 is an excellent opportunity for Australia to shows its commitment and value towards the rare disease community by investing in rare disease translational research.

Rare Diseases when addressed as a collective are not really that rare as a group, and therefore are worth investing in from a health prevention/productivity perspective.

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

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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 pauperization 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.

Rare Voices Australia believes that it is crucial that the rare disease consumer voice has a ‘seat at the table’ and be included at all levels of planning from the development, phase of the Australian Medical Research and Innovation Strategy and Innovation Priorities of projects through to the execution with translational rare disease research. RVA endorses the MRFF Action Group’s call for consumer representation on the Advisory Board.

Kindest Regards,

Megan Fookes
Executive Director
Rare Voices Australia Ltd.