Access to medicines for rare and less common diseases

Roche Australia (Pharmaceuticals) Policy Position

Summary

- The "one size fits all" approach to reimbursement by the Pharmaceutical Benefits Advisory Committee (PBAC) means that medicines for rare diseases often struggle to achieve timely funding and Australian patients experience significant inequality.
- The Australian reimbursement system needs to be reformed to better reflect community values, ensuring that decisions properly consider factors such as rarity, severity and unmet need.
- Roche believes that patient access delays could be resolved by using better evaluation tools that consider clinical, economic and ethical factors; taking a pragmatic approach to evidence; and streamlining processes for low budget impact medicines.

Background

Rare diseases are life-threatening or severely debilitating conditions that occur with low prevalence in the population. Rare Voices Australia defines a rare disease as occurring in less than 5 in 10,000 Australians, and notes that there more than 8,000 rare diseases[1]. Rare Cancers Australia defines a rare cancer as one with fewer than 6 incidences per year per 100,000 population and a "less common" cancer as one with 6-12 incidences per 100,000[2].

Definitions vary, yet what is known is that the less common a condition is, the more it is subject to a number of challenges: poorer diagnosis; greater difficulty in developing treatments and providing evidence through clinical trials; and lack of general knowledge across the clinical community. As all thresholds for rarity are somewhat arbitrary, throughout this document "rare diseases" should be taken to include all conditions, including cancers, where low prevalence poses these challenges.

Roche position

At Roche we are looking to develop innovative medicines that address diseases with unmet medical need and have the potential to revolutionise the standard of care. As a result, Roche is particularly concerned with the ability of patients to access the medicines we create. It can be just as expensive to develop a treatment for a rare condition as for a common one, and yet the costs can only be recouped across a small population. In rare diseases, where under-diagnosis is a problem and patients are geographically-spread, recruitment into clinical trials can be challenging and time-consuming. Data from clinical trials using small numbers of patients can also be more uncertain than larger studies in common diseases.

This poses particular challenges for reimbursement of medicines for rare diseases via the Pharmaceutical Benefits Scheme (PBS), where a high cost per patient combined with uncertainties

in the data will not satisfy traditional economic-based criteria. The Pharmaceutical Benefits Advisory Committee (PBAC) focuses on incremental cost effectiveness ratios (ICERs) and has a low willingness-to-pay for health outcomes that involve "uncertainty". Given the right of all patients, whatever their condition, to access appropriate and effective healthcare, it is important for all stakeholders to work together to resolve these issues. Academics have estimated that Australian rare disease patients wait two to four years longer for similar medicines than patients in Canada, the United Kingdom, Germany and the Netherlands[3].

As many of the issues for rare disease medicines are common to specialised treatments for diseases not considered rare, Roche supports overall reforms to the reimbursement system rather than the establishment of additional funding streams. The necessity of applying eligibility "cut-offs" for a dedicated rare disease medicines program would mean that some medicines for more common but still low prevalence diseases, such as many cancers, may be excluded despite facing the same challenges of "rarer" conditions. In general, Roche supports a flexible system that takes account of where a disease fits on the continuum of rarity.

Roche proposes that the reimbursement system in Australia needs review to ensure it delivers timely access to the medicines Australians need, regardless of the condition they experience. Roche supports a fit-for-purpose evaluation, which would allocate resources and determine processes based on unmet need, complexity and budget impact. Many medicines for rare diseases have limited budget impact and timely access could be improved by simplifying the assessment of these treatments. Some highly-specialised medicines for rare diseases may have a larger budget cost associated and need more complex clinical or economic modelling. In these cases, early engagement of stakeholders and those with the necessary expertise in the disease area is critical.

Roche supports the development of specific considerations for listing of rare disease medicines within the PBS, to be included in the PBAC Guidelines. It is particularly important that a realistic and pragmatic approach is taken to considering the clinical evidence for rare diseases, as it will frequently not be possible to develop "gold standard" evidence in small populations. Appropriate community input into setting assessment criteria for rare or less common diseases is also essential, to ensure that social and ethical factors are considered along with cost and clinical effectiveness.

In the interests of the community and to promote continued innovation in treating rare diseases, Roche encourages stakeholders to work together to resolve these issues.

This position paper was adopted by the Roche Australia (Pharmaceuticals) Leadership Team on 14 November 2017 and entered into force the same day

[1] Rare Voices Australia (RVA). 2015. "Rare Voices Australia Fact Sheet". Sydney

[2] Rare Cancers Australia. "Understanding Rare Cancers", accessed from http://www.rarecancers.org.au/page/1100/rare-cancers, 23/10/17

[3] Robbins A, Lipworth W and Jackson A. 2014. "Funding Rare Disease Therapies in Australia". Health series. McKell Institute, Sydney