Rare disorder updates from Minister's reports sent in July 2018

MINISTER'S REPORT MAY 2018

Medicines for rare disorders

New policy settings

As outlined in an update sent to you on 25 June 2018, PHARMAC has published adjusted policy settings that will underpin our work in funding medicines for rare disorders.

The policy settings include three principles, which are based on the differences of funding medicines for the treatment of rare disorders. These differences address the current market challenges for these medicines, and enable (where appropriate) a different entry into the pharmaceutical funding process. The principles are:

1. The medicine has been approved by Medsafe, or an approved international regulatory authority, for the identified indication.
2. The disorder is a clinically defined disorder affecting an identifiable and measurable patient population with a prevalence of less than 1:50,000 in New Zealand.
3. The medicine is only registered for treating the rare disorder, or if it's registered for other disorders (or is part of phase three clinical trials for other disorders), the cumulative prevalence across all indications still meets the second principle.

Funding applications for medicines for rare disorders

On 29 June 2018, we issued a call for funding applications for medicines for rare disorders (and have informed a number of rare disorders consumer representative groups about this).

We plan on engaging with potential suppliers in Australia and Auckland in July 2018 and with rare disorders stakeholder groups through regular meetings. Our aim is to attract funding applications for medicines for rare disorders, and to ultimately make decisions that will lead to better access to medicines for people with rare disorders.

PTAC Rare Disorders Subcommittee

PHARMAC has approved the establishment of a Rare Disorders Subcommittee of the Pharmacology and Therapeutics Advisory Committee (PTAC). We sought nominations from a wide range of colleges and clinical networks (based in both New Zealand and Australia), and specifically sought applicants who had a special interest in rare disorders. We have appointed 10 expert clinicians as members.

The appointees include some of the country's leading experts in treating rare disorders, and include specialties such as paediatric nephrology, metabolic disorders, haematology and neurology. They also include an Australian specialist in the management of rare disorders.

The Subcommittee includes two PTAC members, to maintain links between this new Subcommittee and our primary clinical advisory committee. They are:

- Professor Tim Stokes, Head of Department - General Practice & Rural Health, Otago Medical School & General Practitioner, Dunedin (appointed Chair)
- Dr Melissa Copland, Pharmacist, Queenstown (appointed Deputy Chair)

Other Subcommittee members are:

- Dr William Wong, Paediatric Nephrologist, Auckland DHB
- Dr Callum Wilson, Metabolic Physician, Auckland DHB
- Dr Dylan Mordaunt, Clinical Geneticist, Auckland DHB
- Dr Janice Fletcher, Clinical Director – Genetics and Molecular Pathology, SA Pathology, Adelaide, Australia
- Dr Humphrey Pullon, Haematologist, Waikato DHB
- Prof Carlo Marra, Dean of the School of Pharmacy, University of Otago, Dunedin
- Dr James Cleland, Neurologist and Neurophysiologist, Bay of Plenty DHB.
- Dr Howard Wilson, General Practitioner, Akaroa.

Treatments for Gaucher disease

The PHARMAC Board has approved a proposal to widen access and change the funded enzyme replacement therapy (ERT) for Gaucher disease.
Around 20 adults and children in New Zealand are currently receiving treatment for this rare disorder. Funded ERT for treating Gaucher disease will change from imiglucerase (Cerezyme) to taliglucerase alfa (Elelyso) and patients will be eligible for a higher maximum dose where clinically appropriate, which could provide greater health benefits for some patients.
The new ERT (Elelyso) will be listed on 1 August 2018, followed by a seven-month transition period during which patients will change treatments with the support of their clinician.

Other treatments

Negotiations are underway on two rare disorders medicines. In early/mid-July, we intend consulting on funding for two listings which treat two different rare disorders – phenylketonuria in pregnancy and Wilson's disease.

Briefings provided in June 2018

"No surprises" briefings – In the last month, PHARMAC has provided you with "no surprises" briefings on:

- *Updated policy settings for rare disorders* – 25 June 2018

MINISTERS REPORT FOR QUARTER 4 2017/18
INCORPORATING MONTHLY REPORT FOR JUNE 2018

Rare disorders
We are progressing our work aimed at improving funded access to medicines for rare disorders. In July 2018, we publicly announced the establishment of a clinical

subcommittee for rare disorders that will provide advice to our main clinical committee, the Pharmacology and Therapeutics Advisory Committee.

We have also been engaging with potential suppliers in Australia and Auckland, and with rare disorders stakeholder groups. Our aim is to attract funding applications for medicines for rare disorders, and to ultimately make decisions that will lead to better access to medicines for people with rare disorders.

Briefings provided this quarter

Rare disorders – progress update on activity underway and planned in the coming months

“No surprises” briefings on:
- Establishment of rare disorders subcommittee
- Updated policy settings for rare disorders
- Calls for nomination for members of a rare disorders clinical subcommittee