Rare disorder updates from Minister's reports (monthly/quarterly):

July 2018

Medicines for rare disorders

Call for applications

PHARMAC has had a good response to our call for applications from suppliers of medicines for rare disorders. During this process, we met with suppliers of medicines for rare disorders in Australia and New Zealand.

The next step is to seek clinical advice on these applications from the newly established rare disorders subcommittee in early November 2018.

Sector engagement

We are progressing the listing of other treatments for rare disorders through our standard process. We continue to work with the Ministry of Health to provide advice where necessary on access to rare disorders treatments. We also continue to meet with key rare disorders stakeholders including the New Zealand Organisation for Rare Disorders and Lysosomal Diseases New Zealand.

Proposal to list sapropterin for women with phenylketonuria

We are currently consulting on a proposal to fund sapropterin, a treatment for the rare condition phenylketonuria (PKU), for women who are pregnant or actively planning a pregnancy and require pharmacological support to manage their PKU in pregnancy. We estimate that about six women each year in New Zealand may be eligible for treatment with sapropterin under this proposal.

August 2018

Medicines for rare disorders

Following our call for applications for medicines for rare disorders earlier this year, we have received 13 funding applications for 10 medicines from eight different suppliers.

The applications will be considered at the inaugural meeting of the Rare Disorders Subcommittee of the Pharmacology and Therapeutics Advisory Committee (PTAC) in early November 2018. Following clinical advice and completion of our assessment processes, including prioritisation, PHARMAC will then determine the next appropriate steps for the applications, such as commercial processes.

Quarter 1 2018-19

Medicines for rare disorders

Following our call for applications for medicines for rare disorders earlier this year, we have received 13 funding applications for 10 medicines from eight different suppliers.

The applications will be considered at the inaugural meeting of the Rare Disorders Subcommittee of the Pharmacology and Therapeutics Advisory Committee (PTAC) in early November 2018. Following clinical advice and completion of our assessment

processes, including prioritisation, PHARMAC will then determine the next appropriate steps for the applications, such as commercial processes.

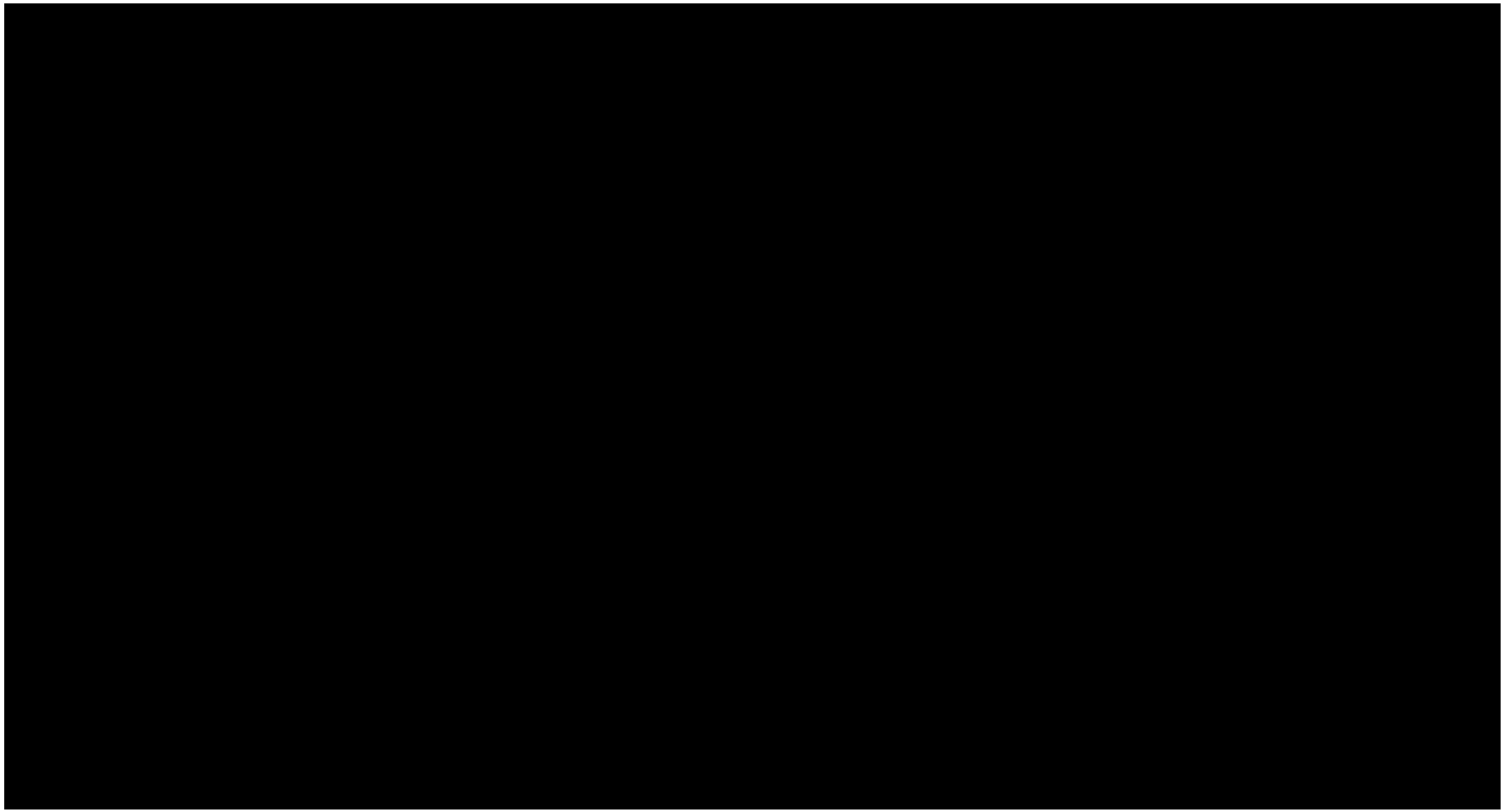
PHARMAC will provide a briefing to you in November with an update on progress to date.

Government expectations

Expectation	Comment
Work on ensuring fair consideration of medicines for people with rare disorders, and in particular, progressing the initiatives identified following our rare disorders pilot.	A set of dedicated features for considering funding medicines for rare disorders has been introduced. A Rare Disorders Subcommittee of PTAC has been established and will meet for the first time in November 2018. *Output 1.1*

Attachment 4 – PHARMAC Board Risk Register, as at 30 September 2018

Important note: the risk register is a considered assessment of the *key risks* facing PHARMAC. It is not a record of (a) all possible or hypothetical risks (of which there are a great number) as part of PHARMAC's normal operating environment; nor (b) risks that are not considered 'key risks' and are being managed as part of PHARMAC's normal business processes. The risks contained in the register are specific risks that PHARMAC considers warrant particularly careful attention and management. Under this approach, there is an inevitable judgement of what is 'in' and 'out', being judgements made by PHARMAC's Management with quarterly review and oversight by the PHARMAC Board.



October 2018

Medicines for rare disorders

Following our call for applications for medicines for rare disorders earlier this year, we have received 13 funding applications for 10 medicines from eight different suppliers.

The applications were considered at the inaugural meeting of the Rare Disorders Subcommittee of the PTAC in early November 2018. Following clinical advice and completion of our assessment processes, including prioritisation, PHARMAC will then determine the next appropriate steps for the applications, such as commercial processes.

PHARMAC provided a briefing in November 2018 updating you on progress to date.

Quarter 2 2018-19

Medicines for rare disorders

The Chief Executive will attend and present on the New Zealand experience at the University of British Columbia (UBC) Summit on Reimbursement and Pricing Policy for Rare Diseases in Vancouver, Canada in March 2019.

The summit will also provide an opportunity to meet with international experts in orphan drug policy, to build networks, and work towards potential solutions to manage this issue.

Thirteen applications (10 medicines) were considered at the inaugural meeting of PHARMAC's Rare Disorders Subcommittee of the Pharmacology and Therapeutics Advisory Committee (PTAC) in early November 2018. Following clinical advice and completion of our assessment processes, including prioritisation, PHARMAC will determine the next appropriate steps for the applications, such as commercial processes.

The Subcommittee meeting minutes have recently been sent to applicants for review and feedback about any content that they wish to be withheld. We are planning to publish the minutes on the PHARMAC website in mid-February 2019. PHARMAC will be engaging with interested people to update them on the recommendations from the Subcommittee prior the public release.

Expectation	Comment
Work on ensuring fair consideration of medicines for people with rare disorders, and in particular, progressing the initiatives identified following our rare disorders pilot.	**A set of dedicated features for considering funding medicines for rare disorders has been introduced. A Rare Disorders Subcommittee of PTAC has been established and met for the first time in November 2018.** **Output 1.1**

January 2019

Medicines for rare disorders

Stakeholder engagement

PHARMAC Board Chair, Steve Maharey and Chief Executive, Sarah Fitt attended the Asia-Pacific Lysosomal Conference in Auckland on 16 February 2019 where the PHARMAC Board Chair presented on PHARMAC's work in funding treatments for rare disorders.

The Chief Executive, Sarah Fitt will attend and present on the New Zealand experience on funding medicines for rare disorders at the University of British Columbia (UBC) Summit on Reimbursement and Pricing Policy for Rare Diseases in Vancouver, Canada in March 2019. The summit will also provide an opportunity to meet with international experts in orphan drug policy, to build networks, and work towards potential solutions to manage this issue.

Rare Disorders Subcommittee

Thirteen applications (10 medicines) were considered at the inaugural meeting of PHARMAC's Rare Disorders Subcommittee of the Pharmacology and Therapeutics Advisory Committee (PTAC) in early November 2018. The minutes of that meeting were published on 12 February and key stakeholders were notified.

The Subcommittee recommended that:

- four of the medicines be funded, for use in hyperammonaemia, hereditary tyrosinaemia type 1, Fabry disease, and cystic fibrosis in patients with a G551D mutation;
- five medicines be declined for funding; and
- one medicine be deferred for re-consideration at a later date following publication of updated clinical trial results.

The next step is for these funding applications to be reviewed by PHARMAC's main body of expert clinical advisors, the Pharmacology and Therapeutics Advisory Committee (PTAC) at its February 2019 meeting.

Publication of medicines for rare disorders

Over recent months, PHARMAC has had a series of enquiries from other jurisdictions about PHARMAC's work in funding medicines for rare disorders, with PHARMAC increasingly seen as taking a leading and innovative approach. In response to this interest, we are developing a report detailing how PHARMAC has approached the challenges many countries are facing in funding medicines for rare disorders. We are considering the most appropriate way to publish this report, including through academic journals.