Consultation

PHARMAC
TE PĀTAKA WHAIORANGA

Proposal to transition funded access to dornase alfa to a standard Special Authority

2 October 2020

What we're proposing

PHARMAC is seeking feedback on a proposal to transition the process for obtaining funded access to dornase alfa (Pulmozyme) from the cystic fibrosis (CF) Panel to a standard Special Authority from 1 December 2020.

The proposal involves the continuation of funding of dornase alpha for eligible patients; the main change would be to the mechanism of application for funding.

In summary, from 1 December 2020 this proposal would result in:

- Access to funded dornase alpha transitioning to a standard Special Authority criteria to be listed in Section B and Part II of Section H of the Pharmaceutical Schedule.

Applications for dornase alfa could be made either electronically, via the electronic Special Authority system, or manually on the Special Authority forms available on the PHARMAC website at www.pharmac.govt.nz/SAForms.

Consultation closes at **5 pm on Friday, 16 October 2020** and feedback can be emailed to cfpanel@pharmac.govt.nz.

What would the effect be?

For Patients

This would involve continuation of funding of dornase alfa for eligible patients. This transition would ensure that those patients who meet the intent of the current criteria for treatment would receive treatment in a more streamlined way. It would also improve equity of access to these treatments as this transition aims to reduce the burden placed on patients in order to access dornase alfa.

The pre pandemic requirement for a stable baseline requiring three spirometry tests over a six-week period would be removed from the initial criteria.

For Pharmacies and DHBs

There would be no significant impacts for pharmacies or DHBs from this proposal

For Prescribers

The proposal would not change those prescribers eligible to apply for dornase alfa. This would however provide a more streamlined mechanism for clinicians working within their vocational scope to apply for funded dornase alfa therapy for their patients. It would also improve equity of

access to these treatments as this transition aims to reduce the burden placed on prescribers when prescribing dornase alfa

Who we think will be interested

- People with cystic fibrosis and their family, whānau or caregivers
- Cystic Fibrosis NZ and any others who support people and families with cystic fibrosis
- People interested in the funding of medicines for cystic fibrosis
- Respiratory physicians, paediatricians, respiratory nurses, and other clinicians and health professionals involved in the management of cystic fibrosis
- Hospital pharmacies and DHBs
- Pharmaceutical suppliers

About Dornase alfa

Cystic fibrosis is a genetic condition that affects the lungs, the digestive system and other organs. It is usually diagnosed soon after birth. There are over 500 children and adults living with cystic fibrosis in New Zealand.

Cystic fibrosis is caused by changes in the Cystic Fibrosis Transmembrane Conductance Regulator (CFTR) protein, leading to thick and sticky mucus that blocks organ function. Symptoms and severity can vary – some people with cystic fibrosis remain well for a long period of time with minimal symptoms or hospital admissions, while others require more intensive medical care. Cystic fibrosis can lead to severe lung damage that is life threatening and eventually leads to death.

Dornase alfa is a medicine used for the management of cystic fibrosis patients to improve pulmonary function. It is delivered by inhalation using a jet nebuliser. Further information regarding dornase alfa can be found on the Medsafe datasheet.

Why we're proposing this

When dornase alpha was originally funded in 1996 there were no satisfactory treatments for cystic fibrosis other than dornase alpha.

Currently members of PHARMAC's Cystic Fibrosis (CF) Panel assess whether applications meet the eligibility criteria for accessing dornase alfa. These arrangements currently target funding for dornase alfa to patients with cystic fibrosis refractory to other treatment options. However, since dornase alfa was funded, the cost of this medicine has reduced considerably compared with other medicines.

PHARMAC is proposing to transition access to dornase alfa to a standard Special Authority. This would provide a more streamlined mechanism for clinicians working within their vocational scope to apply for funded dornase alfa therapy for their patients. It would also improve equity of access to these treatments, as this transition aims to reduce the burden placed on applicants and patients when accessing dornase alfa. In addition, it would also reduce the requirements for patients and their carers/whānau, clinicians and PHARMAC staff when evaluating and managing access to dornase alfa (pulmozyme) on an individual basis.

Details about our proposal

The proposal involves continuing to fund dornase alfa for eligible patients. The proposal would not change those prescribers eligible to apply for dornase alfa. This transition would ensure that those patients who meet the intent of the current criteria for treatment, would receive treatment in a more streamlined way. Accordingly, to ensure this, the transition could involve widening of access to dornase alfa.

PHARMAC does not anticipate any alterations to the group of patients who are currently eligible for funded access as a result of this change in the mechanism of application.

PHARMAC sought advice from the CF Panel and the Respiratory Subcommittee of the Pharmacology and Therapeutics Advisory Committee (PTAC) to develop an appropriate Special Authority criteria to enable this transition. The clinical advisors recommended transferring funded access to dornase alfa to a standard Special Authority that would contain new, but similar, criteria.

There are minor changes proposed to the criteria used by the CF Panel to assess eligibility. The intention of these changes has been to ensure that patients and prescribers are not disadvantaged by the criteria, while ensuring that treatment is made available to those patients considered to meet the intent of the current criteria.

The most important considerations were as follows:

- Removal of the requirement for spirometry to commence treatment with dornase alfa or to renew access to dornase alfa for patients greater than 5 years of age.
- Focussing the criteria on exacerbations rather than hospitalisations for all patients; and
- Generating one set of criteria for all patients with cystic fibrosis.

The proposed Special Authority criteria are as follows:

> **Initial application** - (**cystic fibrosis**) only from a respiratory physician or paediatrican. Approvals valid for 12 months for applications meeting the following criteria:
> All of the following:
> 1.1. Patient has a confirmed diagnosis of cystic fibrosis; and
> 1.2. Patient has previously undergone a trial with, or is currently being treated with, hypertonic saline; and
> 1.3. Any of the following:
> 1.3.1. Patient has required one or more hospital inpatient respiratory admissions in the previous 12 month period; or
> 1.3.2. Patient has had 3 exacerbations due to CF, requiring oral or intravenous (IV) antibiotics in in the previous 12 month period; or
> 1.3.3. Patient has had 1 exacerbation due to CF, requiring oral or IV antibiotics in the previous 12 month period and a Brasfield score of <22/25; or
> 1.3.4. Patient has a diagnosis of allergic bronchopulmonary aspergillosis (ABPA).
>
> **Renewal -** (**cystic fibrosis**) only from a respiratory physician or paediatrican. Approvals valid indefinitely, without need for further renewal unless notified otherwise, where the treatment remains appropriate and the patient continues to benefit from treatment.

All current full supply approvals will be transitioned over to new approvals to ensure continuation of funded access to treatment for these patients.

PHARMAC proposes to transition funded access to dornase alfa from the CF Panel to a Special Authority on 1 December 2020.

To provide feedback

Send us an email: cfpanel@pharmac.govt.nz by 16 October 2020.

All feedback received before the closing date will be considered by PHARMAC's Board (or its delegate) prior to making a decision on this proposal.

Feedback we receive is subject to the Official Information Act 1982 (OIA) and we will consider any request to have information withheld in accordance with our obligations under the OIA. Anyone providing feedback, whether on their own account or on behalf of an organisation, and whether in a personal or professional capacity, should be aware that the content of their feedback and their identity may need to be disclosed in response to an OIA request.

We are not able to treat any part of your feedback as confidential unless you specifically request that we do, and then only to the extent permissible under the OIA and other relevant laws and requirements. If you would like us to withhold any commercially sensitive, confidential proprietary, or personal information included in your submission, please clearly state this in your submission and identify the relevant sections of your submission that you would like it withheld. PHARMAC will give due consideration to any such request.