NEW ZEALAND POMPE NETWORK

8 March 2017

[redacted]

Dear Rebecca

Evaluation of PHARMAC's rare disorders funding pilot

I will be giving feedback particularly to do with the funding process for Myozyme for Pompe disease.

Firstly I would like to ask why Myozyme was considered, and then funded, for infantile Pompe under this RFP, but the same drug for adult onset was considered, and declined funding, under a different pathway? Same drug, same disease, different outcome. New Zealand has **ZERO** infantile Pompe patients. New Zealand has **TEN** adult Pompe patients.

So whilst Pharmac acts like they are trying to fund this rare disease, they have in fact done nothing. Clearly the RFP is nothing more than pretending to pay lip service to rare disease patients with Pompe disease. Therefore, it does NOT work. And, I daresay, designed to that end.

Rather than looking for reasons to DECLINE Myozyme, you need to find a way to FUND people who need the medicine instead of letting us die. How do you propose to do this?

Why do I say you are letting people die? We lost one of our Pompe patients on the 31st of December 2016. Death certificate states that the cause of death was "Respiratory failure due to Pompe disease". This is while we were waiting for the public announcement from Pharmac regarding the decision from the November meeting about whether or not PTAC would recommend Myozyme to be funded for Pompe disease for adults.

If Pharmac were serious about funding medicines for rare diseases, they would have done far more than the pathetic effort they have done. Answer me these questions: **How many patients benefited from the RFP? How many patients still wait for funding for life saving medications?**

I welcome any and all questions from the evaluator regarding my points. I have a lot to say!

NEW ZEALAND POMPE NETWORK

Please also see my responses in the colour red, below.

Seeking your feedback

PHARMAC is seeking feedback specifically from stakeholders who have been involved in the RFP process. We are seeking your views on how well the funding process met the above criteria to feed into the evaluators assessment of the process. We are also interested in your reflections on how PHARMAC ran the process. PHARMAC will be asking the evaluator to provide an objective summary of how well stakeholders viewed the undertaking of this process. The evaluator may choose to follow-up or seek clarity on any feedback you provide if they feel this is necessary.

The questions below are intended to guide your thinking in providing feedback.

1. Please provide your thoughts based on your knowledge and experience in being involved in the RFP, as to how the process met the criteria:

a. How well do you think that the RFP has improved access to effective pharmaceutical treatments has improved?

For Pompe disease there has been ZERO improved access for current Pompe disease patients.

b. How have health outcomes improved for those patients who have received funded treatments via the RFP?

Health outcomes for Pompe disease patients in New Zealand have not improved at all. Funding was given for infantile Pompe disease patients, but we don't have any infantile patients currently.

c. How has the financial risk of running this RFP been managed by PHARMAC?

I don't know the answer to that question. I would suggest auditing Pharmac.

d. How has running this RFP process impacted on the types of commercial proposals for eligible treatments received by PHARMAC, compared to those received in the past?

It hasn't! Companies had the same opportunity in the past to present their treatment options to Pharmac. That hasn't changed at all.

e. Has running this RFP impacted on PHARMAC ability to negotiate good process for the rest of the Pharmaceutical Schedule?

NO, not at all!

NEW ZEALAND POMPE NETWORK

Some comments from an article I did for the local newspaper.

- Way to go Mrs lock you keep fighting I'll sign a petition any time. National you wasted 26 million on a stupid flag yet you won't fund these things so our people can live. On your bike National.
- New Zealand lacks so many modern drugs and treatments! Having travelled extensively and having had first hand experience of medical treatments in other countries, I feel that we are just not up to date, and/or providing much needed drugs in this country- so Pharmac needs to lift its game for the needs of the community!
- New Zealand Gov't & Pharmac, get with the times. These are real people with a great need for help. They deserve your consideration & assistance. Your decision is shameful.
- During Donald Trump's address to Congress, Megan Crowley was applauded as a Rare Disease survivor. She has survived Pompe disease because she has received Myozyme for the last 12 years. It seams that New Zealand sees rare diseases as an inconvenience and puts no value on the lives of its suffering citizens.

As you can see, there is very little to be impressed about regarding Pharmac's faux effort to find a way to fund rare disease medications.

Yours sincerely

Allyson Lock

President

New Zealand Pompe Network

PHARMAC
PO Box 10254
The Terrace
Wellington 6143

7 March 2017

Dear Sir/Madam

Re: Evaluation of PHARMAC's rare disorders funding pilot

I refer to the e-mail dated 15 February 2017 seeking feedback from stakeholders regarding PHARMAC's rare disorders funding pilot. BioMarin responded to the Expression of Interest for Contestable Funds for Rare Disorders in July 2014, [redacted]

[redacted]

In response to PHARMAC's request, BioMarin provides the following feedback:

1. How did the process meet the criteria?

a. Access to effective pharmaceutical treatments is improved:

There was no access to effective treatment for MPS VI prior to the implementation of the rare disorders fund. Galsulfase is the only available treatment for this ultra-rare, severe and chronically progressive life-limiting condition. As such the listing on the Pharmaceutical Schedule is an extremely important step in providing access to effective treatment. Furthermore, if a child is diagnosed in future they would be able to access treatment without delay thereby ensuring optimal long-term health outcomes.

[redacted] This differential access to treatments for ultra-rare diseases results in inequity of access to patients with equal need, and highlights the importance for the New Zealand Government to expand the rare disorders funding program.

b. Health outcomes for those patients who receive funded treatments via the proposal are improved:

It is anticipated that patients who have received treatment with galsulfase via the proposal experience improvements in a range of important health outcomes, including endurance, cardiorespiratory function, and most importantly survival (Giugliani et al.,

2014). Importantly, published studies confirm that the health outcomes of patients with MPS VI are optimised by commencing treatment with galsulfase as early as possible. Hence, the availability of galsulfase through the rare diseases fund is extremely important for any future diagnoses of children with MPS VI (Furujo, Kubo, Kosuga, & Okuyama, 2011; McGill et al., 2009).

c. Financial risk is managed, and expenditure does not exceed the value of the funding provision:

Any financial risk for PHARMAC as a result of the availability of galsulfase through the rare diseases fund is managed [redacted].

d. PHARMAC receives better commercial proposals for eligible treatments than those that have been received in the past:

BioMarin has not previously submitted any proposals to PHARMAC because its therapies for ultra-rare diseases are unable to meet the cost-effectiveness test requirement. [redacted].

e. PHARMAC's ability to negotiate good prices for the rest of the Pharmaceutical Schedule is maintained, for the purposes of securing the best health outcomes for New Zealanders.

Because the RFP had strict eligibility criteria, a defined total budget, [redacted], BioMarin assumes that that there would not be any impact on PHARMAC's ability to negotiate good prices for the rest of the Pharmaceutical Schedule.

2. What was the experience of being involved in the process?

The requirements outlined in the RFP were easy to understand and follow. However, the indicated timelines were not met and BioMarin was not kept well informed about progress of the submitted proposal and subsequently the reasons for the delays in negotiating an agreement. Consequently, any future RFPs could benefit from a more streamlined and transparent process with clear stages and defined timelines. This would help ensure that effective treatments for ultra-rare diseases can be made available more quickly to patients in high need.

Nevertheless, the most important aspect of the RFP for BioMarin was the ability to submit proposals for products that were not registered in New Zealand. It is challenging for a sponsors of effective treatments for ultra-rare diseases to invest significant resources in registering a product with Medsafe, without a viable funding pathway. As such, the RFP addressed this risk and BioMarin assumes that PHARMAC received more proposals as a result.

If you require any further clarification on the information provided, please contact me by telephone [redacted]. We look forward to being advised on the next steps in this process of improving access to life-saving medicines for rare disorders [redacted].

Yours sincerely

Kathryn Evans Country Manager, Australia

References: Furujo, M., Kubo, T., Kosuga, M., & Okuyama, T. (2011). Enzyme replacement therapy attenuates disease progression in two Japanese siblings with mucopolysaccharidosis type VI. Molecular Genetics and Metabolism, 104, 597–602. Giugliani, R., Lampe, C., Guffon, N., Ketteridge, D., Leão-Teles, E., Wraith, J. E., . . . Harmatz, P. (2014). Natural history and galsulfase treatment in mucopolysaccharidosis VI (MPS VI, Maroteaux–Lamy syndrome)—10-year follow-up of patients who previously participated in an MPS VI survey study. American Journal of Medical Genetics Part A, 164(8), 1953-1964. doi:10.1002/ajmg.a.36584 McGill, J. J., Inwood, A. C., Coman, D. J., Lipke, M. L., de Lore, D., Swiedler, S. J., & Hopwood, J. J. (2009). Enzyme replacement therapy for mucopolysaccharidosis VI from 8 weeks of age – a sibling control study. Clinical Genetics, 77, 492-498.

To whom it may concern,

I was involved in the consultation period for listing of bedaquiline for XDRTB so my feedback relates to this.

1. Please provide your thoughts based on your knowledge and experience in being involved in the RFP, as to how the process met the criteria:
a. How well do you think that the RFP has improved access to effective pharmaceutical treatments has improved? *To date I don't believe anyone has accessed bedaquiline in New Zealand.*
b. How have health outcomes improved for those patients who have received funded treatments via the RFP? *NA*
c. How has the financial risk of running this RFP been managed by PHARMAC? *Due to bedaquiline not being listed with Medsafe, the final distribution mechanism had not been finalised on PHARMAC approval. I do now see it has been listed with Medsafe so I expect this process to begin shortly. The feedback given on the financial risk of multiple packs being used for patients and wastage was considered. Given the infectious nature of the disease being treated the financial risk of funding this agent is likely low and the Special Authority/HML criteria are entirely appropriate.*
d. How has running this RFP process impacted on the types of commercial proposals for eligible treatments received by PHARMAC, compared to those
received in the past? *Unsure if any for this product.*
e. Has running this RFP impacted on PHARMAC ability to negotiate good process for the rest of the Pharmaceutical Schedule? *I don't believe so for this product.*

2. Please provide any feedback you may have about your experiences of being involved in the process. For example; was the RFP easy to understand, did you feel you have the opportunity to input into the process, did you feel well informed throughout the process. *I felt well engaged in this process and specific questions were raised directly with the team, I felt this process was more informative and inclusive than the standard consultation process.*

Many thanks

Eamon Duffy
Lead Antimicrobial Stewardship Pharmacist | Pharmacy and Infectious Disease
[redacted]

Dear PHARMAC,
On behalf of the metabolic service I would like to list our thoughts regarding the 'Contestable Fund'.

- Overall, it is great to be trying to streamline the process of funding for rare disease
- We cannot comment on most of the financial aspects of the process
- The outcome has not helped any metabolic patients currently alive. Changing treatments to Special Authority from NPPA has made it easier for the clinician. The process seems like a transfer of cost from one system to another.
- Based on the evidence and need in the country, we do not understand why some decisions were made. For example –
    - Why infantile but not adult Pompe disease was funded
    - Why funding for ERT for MPSII pre and post-transplant was approved (there is no evidence for both transplant efficacy and improved outcomes with idursulfase pre and post, though this generally makes sense)
- It is good to know that we can get ERT for MPSI pre and post-transplant next time we have a new patient. This is likely to occur in the next few years but this is not a major clinical issue and probably won't have much of an impact overall.
- The choice to fund Pheburane was peculiar to us. This was not discussed with us and is not a good product for our patient cohort. It has however been good that the funding of this has resulted in the funding of sodium benzoate, which is safer, used more often and the first line treatment for urea cycle defects. We have also been able to get NPPAs for sodium phenylbutyrate for the patients that need this and don't tolerate Pheburane.
- Funding for MPSVI is probably good and will likely help young children with this if we get them, but this is a very rare disease.

Moving forward, in terms of helping the metabolic community more, as previously discussed with PHARMAC, we would really like to see money and time spent on some of the following:

- More discussion with medical teams about what is needed for patients
- Approving orphan medications for patients' that exist in the NZ system
- Improving protein substitute choice for PKU patients
- Funding sapropterin for more PKU patients
- Including all amino acid disorders (e.g. GA1, tyrosinaemia) in the Loprofin food special authority – not just PKU, homocystinuria, MSUD
- Making Polycal lifetime special authority for metabolic patients, as it is for CF
- Finding and good, reliable source of L-carnitine and listing as special authority
- Listing life-saving vitamins such as riboflavin and biotin for use in metabolic patients on special authority.

Thank you for the opportunity to voice our feedback.

Kind regards,

[redacted] (on behalf of the National Metabolic service)

[redacted]

Dear Rebecca,

Thank you for asking me my view on this. I will take heed of your questions below but summarise within one paragraph.

I don't think the process was valuable at all. It led to funding therapies that were of very low efficacy and extremely poor cost per QALY. From my perspective it has not "improved the overall health of NZers within the available resources" but given access to a small grouping of individuals with very poor cost effectiveness. The process did not achieve gains that could not have been made by standard processes. Even the access to the MDR Tb therapy could easily have been undertaken by a standard process.

I would strongly discourage repeating this process in the same format in the future.

Regards

Graham

The questions below are intended to guide your thinking in providing feedback.
1. Please provide your thoughts based on your knowledge and experience in being involved in the RFP, as to how the process met the criteria:
a. How well do you think that the RFP has improved access to effective pharmaceutical treatments has improved?
b. How have health outcomes improved for those patients who have received funded treatments via the RFP?
c. How has the financial risk of running this RFP been managed by PHARMAC?
d. How has running this RFP process impacted on the types of commercial proposals for eligible treatments received by PHARMAC, compared to those received in the past?
e. Has running this RFP impacted on PHARMAC ability to negotiate good process for the rest of the Pharmaceutical Schedule?

Dr Graham Mills |General Medicine & Infectious Diseases Consultant |p [redacted]

Good morning,

Thank you for the opportunity to provide feedback on rare disorders RFP/Contestable Fund trial.

From our perspective as a patient organisation we can offer valuable feedback on 1a and b, and 2 for the introduction of Firazyr as follows:

1. Please provide your thoughts based on your knowledge and experience in being involved in the RFP, as to how the process met the criteria:

a. How well do you think that the RFP has improved access to effective pharmaceutical treatments has improved?

b. How have health outcomes improved for those patients who have received funded treatments via the RFP?

For HAE patients, the impact of having a life saving treatment available to them on their person is nothing short of life changing. Patients have reported to us a significant increase in their quality and enjoyment of life for both themselves and those around them - their carers, families, businesses and colleagues.

There is a real benefit to treating an HAE attack early, preferably in the first 1-2hrs of symptoms appearing. Treating at this time means the attack can subside quickly in hours rather than days.

Having quick acting treatment available supports not only patients but the support people in the patients life who often carry the burden during an attack that can cause the sufferer to be unable to contribute to their home life, workplace or school.

Being able to treat at the immediate onset of an attack at home or wherever they are means that these patients can continue their normal activities and lives. It also removes the significant anxiety of having a life threatening attack and not being close enough to treatment.

Having access to home treatment also means patients do not have to present at the emergency department and therefore saves time and healthcare costs in reduced hospital admissions.

2. Please provide any feedback you may have about your experiences of being involved in the process. For example; was the RFP easy to understand, did you feel you have the opportunity to input into the process, did you feel well informed throughout the process.

The RFP process was very well managed from our perspective. We were sought out and engaged by Pharmac and kept involved throughout the process. We were well informed and communicated with.

Please don't hesitate to contact me should you have any further questions.

Kind regards,
Olivia

Olivia Worthington
Director

HAE Australasia Ltd
[redacted]

HAE Australasia
hope • advocacy • education
Australasian Patient Organisation for Hereditary Angioedema

REGISTERED CHARITY

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6 March 2017

Rare Disorders Funding Pilot

The mission of Cystic Fibrosis New Zealand (CFNZ) is to optimise quality of life for people with cystic fibrosis and their families – striving for normal life expectancy.
Our organisation supports around 500 children and adults with cystic fibrosis (CF) and their families by providing a wide range of care from fieldworker assistance, information, welfare grants to providing nebulisers and advocating on behalf of the CF community.
One of CFNZ's goals is to negotiate with Pharmac for feasible funding options to make all key CF medications and treatment that are available globally available in New Zealand, with a view to achieving world class standards.

Thank you for the opportunity to respond to Pharmac's Rare Disorders Funding Pilot.

Cystic fibrosis medications were not used for the pilot however Cystic Fibrosis New Zealand can see it was a worthwhile exercise and we note some benefits have resulted including the addition of more medication for rare diseases to the Pharmaceutical Schedule.

We would like to provide some general points in relation to how this system might affect CFNZ and also from feedback speaking to other rare disease organisations.

Cystic fibrosis is generally classed as an orphan disease but the majority of our community don't meet the requirement for rarity under this pilot criteria. CFNZ believes the funding pool should be widened to include all genetic types of cystic fibrosis conditions.

There are some rare forms of CF, including those who have the G551D gene which affects 30 New Zealanders (about one in 156,000 people in NZ), which are likely to meet the criteria for "ultra-orphan" status. However, the medication ivacaftor, which helps to repair the mechanism of the faulty G551D gene, remains unfunded in New Zealand. We know that negotiations with the manufacturer Vertex have stalled. Even if this genetic form of cystic fibrosis did obtain funding from the contestable fund, $5 million a year would not be enough to cover treatment costs.

One of CFNZ's biggest concerns with a policy to foster increased competition between pharmaceutical companies is that it while it sounds promising in theory it is unlikely to improve access to new medications for cystic fibrosis and other rare diseases because often there is no competition in the market.

Vertex, which also produces lumacaftor, that could treat a large percentage of people with CF, has no direct competitors at this stage nor do they have a range of medicines for other disorders to help bargain with. A generic medicine for these products is not on the horizon. Products like pulmozyme, which is manufactured by Roche and has been available since

1993, does not have a generic competitor due to laws surrounding the manufacture of biologic medication, so increasing competition to bring down prices is probably not going to be much assistance in this instance.

The CF community is particularly dissatisfied with the amount of funding for rare medicines given the level of need there is in the rare disorders community. We do support moves to ring-fence funding for rare medicines but again, would like to see all forms of cystic fibrosis included in this funding pool.

We understand there's some uncertainty about the future of this funding pool and there's a desire from patients to get it permanently established. There is added concern about lack of clarity around decision-making criteria and that not enough patients have benefited from the pilot.

CFNZ sought feedback from the New Zealand Organisation for Rare Disorders about their experience with the Funding Pilot. They noted a lack of transparency about the application process and have called for a dedicated rare disorders PTAC subcommittee to be established. It's also important to CFNZ that the relevant clinicians are consulted for applications.

CFNZ appreciates Pharmac's work trialling new methods to manage the pharmaceutical budget and we thank you for being kept informed about ongoing developments.

Kind regards,

Jane Bollard
Chief Executive
Cystic Fibrosis New Zealand

Hello Jude and Rebecca,

[redacted] So I cannot devote the planned time to refining the comments for a submission on this pilot, from Lysosomal Diseases New Zealand.

Here are my rough notes, largely unedited which I'd like you to put into the evaluation. Please advise the external evaluator that I'd like an opportunity to discuss these points with them, as they are "unpolished" and so do not necessarily convey all the key messages as precisely as I'd like.

- There is a need to address Pharmac's credibility in how it presented this fund in media statements. The total available as new spending was $5 Million, not $25 Million. $5 Million has been spent and it is gone. No new investments can be made under this fund unless a new funding allocation is made. $25 Million is a "smoke-and-mirrors" figure that comes about by counting five years ahead, and getting the total that will be spent by maintain $5 Million initially spent, in 4 subsequent years.
- It was a good thing that Pharmac did to set this up, to test a way of getting better access to orphan drugs, because there has been significant unmet need. And the gains from this fund are very positive for patients who are now getting treatment. But it is not a solution to the unmet need.
- The criteria were broadly OK, (catering at 1 in 50,000 delivering for patient groups up to 110 in NZ), but any cut-off was bound to create anomalies at the margins for groups slightly over that number. A better approach would be to have a threshold based on a combination of rarity, availability or not of effective treatments, and severity of disease impacts.
- The process was very long and unacceptably drawn out. A degree of urgency should have been applied.
- It is notable that of the original list of potential therapies listed in the original RFP (which we estimate covered more than 100 patients), we estimate fewer than 5 patients with those diseases will gain access under this pilot, to treatments that were not previously available from any publicly funded source (including NPPA).
- It is clear the total budget allocation under this pilot was far from adequate to make any more than a dent in the issue. Early claims by advocacy groups that about $20 to $25 Million per annum were needed to deliver on the bulk of the needs for rare disorders, seem vindicated by the figures we can now see. We had assumed significant discounts negotiated in arriving at that figure.
- Note that much of the expenditure is that which comes from counting new expenditure on medicines transferred out of the NPPA system to the schedule. The easier access is good but that disguises transferring between pools rather than new access to previously unfunded medicines.
- The evaluation criteria:
    a. Access to effective pharmaceutical treatments is improved;
    b. Health outcomes for those patients who receive funded treatments via the proposal are improved;
    c. Financial risk is managed, and expenditure does not exceed the value of the funding provision;

d. PHARMAC receives better commercial proposals for eligible treatments than those that have been received in the past; and
e. PHARMAC's ability to negotiate good prices for the rest of the Pharmaceutical Schedule is maintained, for the purposes of securing the best health outcomes for New Zealanders.

are very narrowly focused on the systems, processes and costs issues of Pharmac, with little or no focus on the equity, right to health, and fairness issues which should be important considerations. (The fund came about in response to considerable dissatisfaction LDNZ and other advocacy groups with systems that were marginalizing these considerations).

- In spite of that, yes there will be (very slightly) improved access, and better health outcomes for those treated. We expect the budget will be managed because that is one thing Pharmac seems to do very well. The pilot did seem to generate some additional proposals, including from new (to NZ) suppliers so that is a very positive thing also.
- The final evaluation point about negotiating good prices for the rest of the schedule has "hooks" in it. From a patient advocacy perspective, this is probably the weakest link in the policy and appears to ensure relative cost-effectiveness with rare and common drugs is maintained as a central tenet of the scheme. Therein lies the contradiction, highlighted it would seem by PTAC's advice on Myozyme (admittedly outside of this pilot process) which shows little or no consideration for the inherent disadvantage of rarity and maintains a narrow focus on CUA as the major driver of decisions. That is unacceptable in terms of the right to health, and consideration of equity and fairness, and community values. This is not consistent with some of the stated or implied messages about the intent of this special fund.
- A better approach would be to have a more adequate budget, ringfenced from any comparison with drugs for more common diseases, with a wider set of considerations as to criteria for inclusion (rarity, severity and availability/effectiveness of other therapies), the specific inclusion of right to health, equity, fairness and community values in the decision making factors, and preferably managed by a team that doesn't get conflicted by also managing general medicine budgets.
- Whatever the approach, we would encourage a more generous allocation of funds to better meet outstanding needs, with a commitment to moving more promptly with the whole process.
- Conclusion: The pilot has done very little to make a dent in the medicine needs of patients with rare diseases. Note our estimates of the actual "new" treated patients versus those indicated as potentials in the RFP. When this pilot and the PTAC recommendation of Myozyme are taken together, and considered in the context of the new "factors for consideration" it seems inescapable that Pharmac has made almost no real and substantive changes to any of its policies, procedures and outcomes.

I hope this rough set of notes is helpful to your review and I look forward to a chance to discuss this with the external reviewers.
Regards, john

John Forman
[redacted]

Muscular Dystrophy
New Zealand

6 March 2017

Rebecca Elliot
Senior Policy Analyst
PHARMAC

Dear Rebecca

Re: Feedback on PHARMAC's rare disorder funding pilot

We are providing feedback on behalf of the Muscular Dystrophy Association of New Zealand (MDANZ), which is a member led organisation representing over 2000 members nationwide living with neuromuscular conditions, the majority of which are rare disorders. MDANZ aims to ensure individuals affected by these conditions have access to pharmaceuticals for the treatment and management of their conditions.

Firstly, we wish to applaud PHARMAC for taking the necessary steps to establish a contestable fund specifically to improve access to high cost medicines for rare disorders.

Secondly, we are supportive of the work done to facilitate funding of medicines for rare disorders and we also wish to advocate for a continuation of, and an increase in funding of the orphan drugs funding pool as there remains unmet need within this population. We urge you to consider how any extra funding given to PHARMAC in through the government budget can be utilised for people with rare disorders.

In response to the evaluation criteria we are unable to comment on criteria c, d and e as we are not cognisant of this level of detail of PHARMAC's operations however our responses to 1.a,b and 2 are detailed below:

1.a We believe the Request For Proposals (RFP) has improved access to effective pharmaceutical treatments and we would like to see this rate of improvement increase.

We wish to note that in addition to directly benefiting individuals with rare conditions, increasing funding in this pool has achieved the secondary benefit of attracting pharmaceutical companies back to the New Zealand market, who would otherwise have not had an opportunity to apply to PHARMAC for funding for orphan drugs.

Muscular Dystrophy New Zealand

1.b We have not had any direct experience of how health outcomes have improved for those patients who have received funded treatments via the RFP as none of the treatments listed are for people with neuromuscular conditions, with the exception of the listing of Alglucosidase alfa (Myozyme) for infantile-onset Pompe Disease (listed from 1 December 2016). We are reassured and relieved of significant concern and anxiety with the listing of Myozyme for infantile-onset Pompe disease, however we are, obviously, unable to comment on whether this has improved health outcomes as at the time of writing there are no patients within New Zealand with infantile-onset Pompe disease. It is an ongoing concern for the adult population with Pompe disease, that they have been denied funded access to Myozyme via PHARMACs process.

2. Overall, our experience of being involved in the process has been positive. We think that the RFP is relatively easy to understand, however we think that by increasing the number of points in the process where the community-based support organisations are contacted and / or provided with information or opportunities for input should be increased. We note that at times the information provided about the RFP has been ambiguous and has led to misunderstandings and the need to seek further clarification from amongst community-based support organisations.

In addition, we highlight that the high threshold set for what PHARMAC considered 'rare' in its defining criteria means that some rare and potentially severe conditions miss out. We also wish to see the application process happen within shorter time frames due to the sense of urgency and the nature of these conditions.

Our final comment is regarding the formula for assessing the cost effectiveness of a treatment. This should be more flexible in the instance of rare disorders, where small numbers of recipients may benefit from a high amount of investment. This is fundamentally different from a high volume/population based funding model.

Thank you for the opportunity to give feedback at this time. We are happy to be contacted for further input in the future.

Yours sincerely

Ronelle Baker
Chief Executive

Miriam Rodrigues
Programme & Service Advisor

419 Church Street East, Penrose, Auckland 1642 | P O Box 12063, Penrose, Auckland 1061
0800 800 337 | info@mda.org.nz | www.mda.org.nz

Charity Registration Number CC31123

[redacted]

23 February 2017

To the PHARMAC evaluation team,

I am responding to your request for feedback regarding the RFP process.

[redacted] is on Firazyr for HAE.

a. How well do you think that the RFP has improved access to effective pharmaceutical treatments has improved?
[redacted] is now able to get this medication on a regular basis. This has reduced the stress associated with not knowing whether or not [redacted] use of Firazyr was going to be continued, after [redacted] had had a small trial of it and found it meant a lot less hassle and time spent in hospital.

b. How have health outcomes improved for those patients who have received funded treatments via the RFP?
The availability of Firazyr for the treatment of [redacted] HAE has increased [redacted] quality of life. [redacted] no-longer has to go to the hospital every month for treatment of acute attacks as the Firazyr allows [redacted] to treat most of these at home. This also reduces the stress associated with an attack and having to negotiate the rigours of A&E.
Having [redacted] acute remedy at home/readily available has also meant [redacted] can travel around the country without having to worry about hospitals in small areas carrying the other acute medication Berinert P.
[redacted] does still occasionally need to use Berinert P, when [redacted] is having a particularly bad swell and nothing else will reduce the swelling. This happens maybe twice a year. Before the Firazyr became available [redacted] was having to go to A&E for an infusion of Berinert P every month.

c. *How has the financial risk of running this RFP been managed by PHARMAC?*
I can't really comment on this but am aware that the cost of the Firazyr is less than the cost of Berinert P and the associated cost of having to visit A&E for the administration of it.

Thank you for the chance to be able to comment on this process. I have found it beneficial to be part of the process and to have our voices heard in regard to getting better treatment and an improvement in quality of life for people with this rare disease of HAE.

[redacted]

File note 9 February 2017

Meeting with NZORD and PHARMAC re rare disorders pilot evaluation

Attendees: Letitia O'Dwyer (NZORD), Jude Urlich, Angela Mansell, Rebecca Elliott (PHARMAC)

Meeting notes

- Patient groups that NZORD represent have noted that the turn-around time for funding decisions through the RFP process was lengthy.
    - Suggestion was made by NZORD to represent the process and time taken for applications through a flow-chart diagram showing different stages as part of the evaluation (eg RFP close, clinical advice obtained, ranking completed, negotiations completed, consultation period, decision notified, listing date), and dates Medsafe registration applied and approved (if relevant).

- Some patient groups represented by NZORD felt that there was a lack of transparency from PHARMAC around where in the process certain funding applications, through the RFP, were. The importance of personalised feedback to particular patient groups was reiterated. It was acknowledged that this did take place with some patient groups as part of this process.

- Some patient groups were unclear why some rare disorders received funded treatments yet others with comparatively larger patient populations (albeit still rare) were not. PHARMAC noted that it was not possible to release information as to whether bids were received for those, and that it may be difficult for an evaluator to make any comment in that respect.

- NZORD suggested the role of a rare disorders PTAC sub-committee on an ongoing basis would be beneficial, noting the specialised nature of rare disorders and their potential treatments.
    - It was noted that there was a lack of visibility about the composition of clinical expertise in committees
    - It was suggested by NZORD that external clinical advice could also be bought in to provide clinical advice where appropriate and necessary
- PHARMAC noted that the evaluator could be asked to include information on the composition of the clinicians who provided advice throughout the process.

- NZORD noted the potential benefits of the RFP was that new suppliers to NZ were bought into the market, with potentially larger rare disorders product portfolios than currently available for future funding applications. PHARMAC noted that while this would not be a benefit of the RFP itself, it could possibly be noted by the evaluator as a consequential benefit overall.

- NZORD suggested that PHARMAC could have communicated more effectively to patient groups about the process and why decisions were made.
    - PHARMAC noted the trade-off to be made around transparency and commercial sensitivity in such commercial negotiations (eg. cannot reveal all of the products where commercial bids were made, nor the

names of the companies that were not conforming). PHARMAC welcomed any comments on that aspect. It was noted that the evaluation might usefully explain the differences between a funding application where the name of the supplier, the product and the indication are all identified at the point of application; a regular RFP process where even the presence or absence of bids is confidential and there is no awareness until consultation on a specific provisional agreement occurs; and the rare disorders RFP which provided for the names of suppliers but not the products nor the total number of bids per supplier (although the total number of bids overall was released).

- NZORD asked about the allocation of funds for rare disorders in the future, noting the $25m over five years as part of this process, and the visibility of this for the Minister.
    - PHARMAC outlined that there are no ring-fenced funds and the additional money is part of PHARMAC's combined pharmaceutical budget
    - It was noted that the Minister is aware and will be informed of the outcome of the evaluation. It was also noted that the PHARMAC Board is the decision-maker.

- PHARMAC noted there was a risk that stakeholders may perceive the evaluation to be deficient if it did not include any information on 'what next?' NZORD noted that it would be important to ensure that people were very clear as to the nature of the evaluation.

To whom it may concern,

I would like to provide feedback on the rare disorders funding pilot - my experience is as a Hereditary Angioedema patient. My feedback to the questions I can answer are below:

1. a. Access to effective pharmaceutical treatments is improved:
Firazyr (Icatibant) injections are now available to eligible HAE patients through their specialist/immunologist. This is a huge leap forward for Hereditary Angioedema patients who can now carry treatment with them rather than go to a hospital emergency department and wait to be seen and hope they stock C1 INH concentrate. I [redacted] It will be a breakthrough for me personally to know that I can carry this with me wherever I go and no longer have the stress and anxiety of finding myself somewhere without access to treatment. Once I am able to take Firazyr I am looking forward to traveling and doing activities that were previously too risky to participate in as I am nervous of being too far from the hospital.

1. b. Health outcomes for those patients who receive funded treatments via the proposal are improved:
Other patients that I know have seen a significant improvement in the length of their attacks and needed less hospital visits. I'm looking forward to experiencing these benefits myself in the coming months.

2. I have found the process to be transparent with excellent communication for us as patients. It was good to be able to provide a submission in support of the funding and also feedback on the process as a whole. Pharmac staff were very helpful throughout and I felt supported through the process. This was much better than I had expected.

Thank you,
[redacted]

Hi,

Please find below my feedback.

The questions below are intended to guide your thinking in providing feedback. - Icatibant (Firazyr) for hereditary angioedema (listed from 1 January 2016)

1. Please provide your thoughts based on your knowledge and experience in being involved in the RFP, as to how the process met the criteria:
   a. How well do you think that the RFP has improved access to effective pharmaceutical treatments has improved?

I think the RFP has dramatically improved access to Icatibant, in that it can be ordered from the local pharmacy. Previously it was either not available or only from the hospital pharmacy.

b. How have health outcomes improved for those patients who have received funded treatments via the RFP?

[redacted] has been able to lead a relatively normal life, not being scared to go on holiday where [redacted] is a long way from the hospital. Before having access to Icatibant, [redacted] would be in the ED monthly (every 3 weeks)
It means that [redacted] can treat herself at home and not worry about how to get to the hospital, and who will look after the kids, especially in the early hours of the morning.
It has removed some of the fear of a throat swell.

c. How has the financial risk of running this RFP been managed by PHARMAC?

I have no views on this.

d. How has running this RFP process impacted on the types of commercial proposals for eligible treatments received by PHARMAC, compared to those received in the past?

I have no views on this

e. Has running this RFP impacted on PHARMAC ability to negotiate good process for the rest of the Pharmaceutical Schedule?

I have no views on this

2. Please provide any feedback you may have about your experiences of being involved in the process. For example; was the RFP easy to understand, did you feel you have the opportunity to input into the process, did you feel well informed throughout the process.

I found the RFP process really easy. The documentation was clear and concise.
I felt really good being asked to take part in the process. I felt listened to and that my feedback was valued.
It was a good experience and gave my family and I an insight into the process.
The whole process was transparent and the staff I made contact with were really nice and professional.

Regards
Shane Burke

[redacted]

[redacted]

[redacted]