Department of Haematology
Waikato Hospital
[redacted]

29 July 2013

Ms. Sue Anne Yee
Therapeutic Group Manager
PHARMAC
PO Box 10 254
WELLINGTON 6143

Dear Sue Anne,

Re: Feedback submission on PHARMAC's proposal to decline a funding application for Eculizumab

Please find attached my feedback on the above proposal.

In addition to the attached written material enclosed, I would also request that I be given the opportunity to discuss this feedback face-to-face, as outlined on Page 2 of the PHARMAC Consultation Document of 21 May 2013.

Thanking you,

Yours sincerely

HUMPHREY PULLON

Dr. Humphrey Pullon
MBChB, FRACP, FRCPA
CONSULTANT HAEMATOLOGIST.

EXECUTIVE SUMMARY

I strongly oppose the PHARMAC proposal to decline a funding application for eculizumab for the treatment of New Zealand (NZ) patients with Paroxysmal Nocturnal Haemoglobinuria (PNH) because:

1. Eculizumab is a highly effective treatment for PNH.

2. Eculizumab dramatically improves the long term survival and quality of life of PNH patients.

3. PHARMAC has not consulted with either NZ or international haematologists who have experience in treating PNH patients with eculizumab, and therefore does not appreciate the dramatic benefits of eculizumab treatment.

4. The number of New Zealand patients with severe, haemolytic PNH who really require treatment with eculizumab is relatively small. What's more, not all PNH patients need to remain on treatment with eculizumab long-term.

5. Eculizumab could just be made available to the small number of NZ PNH patients with the more severe, haemolytic type of PNH using clearly defined but restrictive NZ access criteria

6. The potential cost of eculizumab to PHARMAC is much less than has been quoted.

7. PHARMAC does not have a suitable process for the transparent and fair evaluation of high cost medicines used for the treatment of rare diseases. PHARMAC needs to address this, and if necessary should evaluate eculizumab via such revised criteria.

1. Eculizumab is a highly effective treatment for PNH.

Eculizumab has been used to treat PNH since 2003, and is now a funded treatment for PNH patients in almost all of the OECD countries, with the exception of NZ.

There is now overwhelming evidence of the effectiveness of eculizumab in the treatment of patients with PNH. Instead of PNH patients suffering from a progressive, debilitating disease, treatment with eculizumab allows them to return to having full and active lives, with the prospect of having a much longer survival.

Eculizumab is a highly effective treatment for patients with severe PNH because:

a). It causes a marked reduction in red blood cell haemolysis
This was first noted in the eculizumab pilot study published in the New England Journal of Medicine in 2004[1]. In treating those first 11 PNH patients, it was soon noted that after only one or two doses of eculizumab, the red blood cell haemolysis was dramatically switched off, and the serum LDH levels plummeted to normal or very near normal levels.

As a consequence of the much reduced red cell destruction, the requirement for regular red cell transfusion support also diminishes dramatically. This was shown in a formalized way in the TRIUMPH study, published in the New England Journal of Medicine in 2006[2]. In this study 87 transfusion-dependent PNH patients underwent randomisation to either receive treatment with eculizumab over a 26 week period or not. Eculizumab reduced ongoing haemolysis by a median of 85.8%. During the study period, 49% of the eculizumab-treated patients did not require any red cell transfusions at all, whilst the remainder had a very much reduced transfusion requirement.

Subsequent studies have shown that in many cases eculizumab treatment removes the requirement for red blood cell transfusion completely[3].

b). It markedly reduces the depletion of nitric oxide, which results from the presence of free haemoglobin in the circulation. As a result of this, the severe fatigue associated with PNH improves, and disabling abdominal pain, caused by muscle spasms and cramping resolves.

This issue was well studied in the SHEPHERD study, published in Blood in 2008[3]. In that study involving 97 PNH patients, all of whom were treated with eculizumab, careful attention was paid to patients' symptoms both prior to and following eculizumab treatment. That study showed a dramatic reduction in abdominal pain, back pain, muscle spasms, diarrhoea and headache with eculizumab treatment. That improvement occurred promptly, after only one or two weeks of eculizumab treatment, and was sustained throughout the study period.

The SHEPHERD Study also looked carefully at patients' fatigue scores, using the FACIT-Fatigue instrument and the EORTC QLQ-C30 fatigue scale. Both scoring systems showed a marked improvement in patients' well being, with improved scores being noted from week 1 of eculizumab treatment. This improvement was also sustained throughout the study period.

The depletion of nitric oxide that occurs in PNH patients also contributes to dyspnoea and pulmonary hypertension. The latter results in significant right heart strain, and an increased risk of pulmonary emboli. Almost 50% of untreated PNH patients have elevated BNP levels as a result of this. Treatment with eculizumab, even relatively late in life, results in a significant reversal of this potentially life-threatening complication. Eculizumab treatment dramatically reduces NO depletion, which leads to a fall in BNP levels and reduced pulmonary artery pressures. This change is associated with improved dyspnoea scores[4, 5].

c). It markedly reduces the risk of developing thrombotic complications. This is especially important, since major thrombosis and the complications thereof is the major cause of death in PNH patients[6].

The issue of a markedly increased risk of thrombosis has been extensively studied in PNH patients, both in those who have not and in those who have received eculizumab treatment[7, 8]. It is now clear that the complement and coagulation systems are closely integrated, with each influencing the activity of the other. Indeed thrombin itself has recently been shown to activate the alternative pathway of complement[9].

Pathogenic factors seen in PNH that contribute to the markedly increased thrombotic risk include: abnormal platelet activation, the toxicity of free haemoglobin, NO depletion, the absence of other GPI-linked proteins on platelet membranes, and abnormal endothelial function. In addition a number of other complement-mediated pro-coagulant mechanisms have been identified, including the pro-inflammatory effects of C5a[8].

Treatment with eculizumab, which switches off the complement dysfunction and its consequences, markedly reduces the risk of thrombosis in PNH patients[7]. Eculizumab treatment results in a rapid and sustained decrease in markers of thrombin generation and inflammation, independent of its effects on haemolysis[10].

Long term follow-up of a large cohort of eculizumab-treated patients from the TRIUMPH and SHEPHERD studies shows that the administration of eculizumab reduced the thrombo-embolic incidence rate from 11.13 events per 100 patient-years to 2.14 events per 100 patient-years. This was a relative reduction in thrombosis risk of 81.8%[11]. Long term follow-up of the UK Eculizumab-treated PNH cohort of 79 patients showed that whilst 27% of the patients had had a thrombosis prior to starting eculizumab (5.6 events per 100 patient-years), only 2 thrombotic episodes developed whilst on eculizumab (0.8 events per 100 patient-years) $p<0.001$[12]. This is a dramatic reduction in thrombotic risk that is directly translated into improved long term survival (see below).

In addition, knowing that eculizumab is highly protective of developing a thrombosis has allowed some PNH patients to safely discontinue primary thrombosis prophylaxis with Warfarin. In the UK cohort, 21 PNH patients with no prior history of thrombosis have discontinued Warfarin after starting eculizumab with no thrombotic sequelae[12].

Treatment with eculizumab therefore allows patients to live their lives without the constant threat of developing a major thrombosis that might severely threaten major organ function or cut their life short. The prospect of being able to safely discontinue

Warfarin therapy in some PNH patients who are on eculizumab is very attractive, and would undoubtedly impact on patient freedom and quality of life.

I am very disappointed to read in the PHARMAC Consultation Document that "...there is evidence that there are some clinical benefits from eculizumab treatment…".
I would argue that there is overwhelming evidence that there are many clinical benefits from eculizumab treatment. Indeed **eculizumab treatment results in a reduction of every adverse clinical outcome associated with PNH**, with the exception of severe bone marrow aplasia and transformation to acute leukaemia[11]. The health gains for any individual, severe PNH patient treated with eculizumab are HUGE.

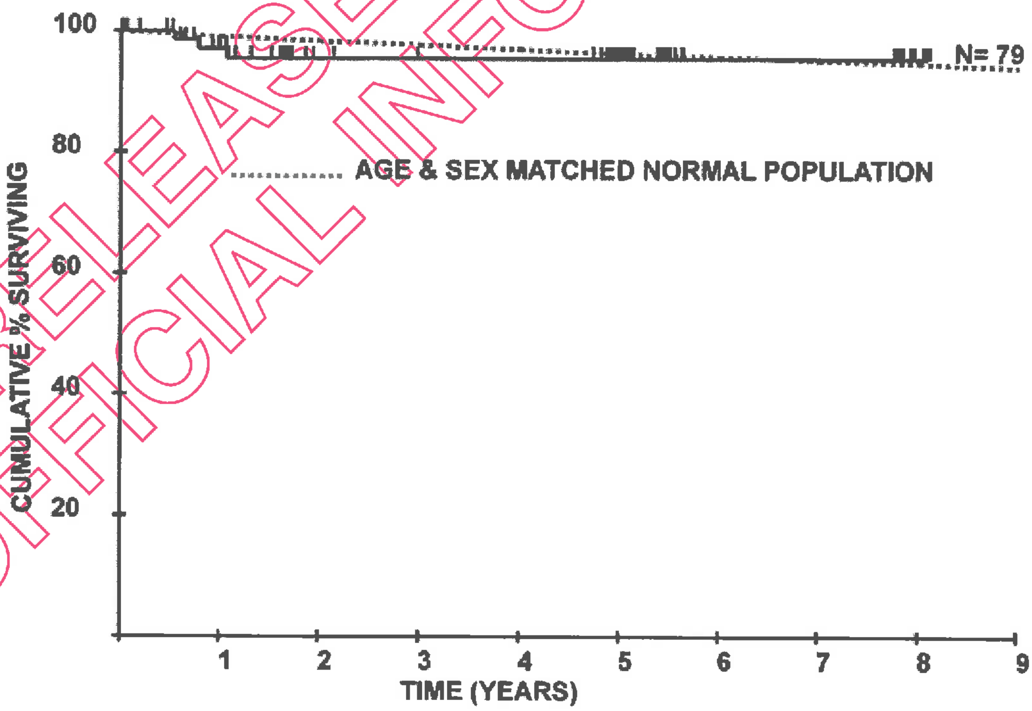
References:
1. Hillmen P., Hall C., Marsh J. et al. NEJM 2004, 350, 552-559.
   Effect of eculizumab on hemolysis and transfusion requirements in patients with paroxysmal nocturnal hemoglobinuria.
2. Hillmen P., Young N., Schubert J. et al. NEJM 2006, 355(12), 1233-1243.
   The complement inhibitor eculizumab in paroxysmal nocturnal hemoglobinuria.
3. Brodsky R., Young N., Antonioli E., et al Blood 2008 ,111 (4) 1840-1847.
   Multicenter phase 3 study of the complement inhibitor eculizumab for the treatment of patients with paroxysmal nocturnal hemoglobinuria.
4. Hill A., Rother R., Wang X., et al., Br J Haematol 2010, 111 (10) 1-12.
   Effect of eculizumab on haemolysis-associated nitric oxide depletion, dyspnoea, and measures of pulmonary hypertension in patients with paroxysmal nocturnal haemoglobinuria.
5. Hill A., Sapsford R., Scally A., et al., Br. J Haematol 2012, 158(3) 409-414.
   Under-recognized complications in patients with paroxysmal nocturnal haemoglobinuria: raised pulmonary pressure and reduced right ventricular function.
6. Hillmen P., Lewis S., Bessler M., et al., NEJM 1995, 333 (19), 1253-1258.
   Natural history of paroxysmal nocturnal hemoglobinuria.
7. Hillmen P., Muus P., Duhrsen U., et al., Blood 2007, 110 (12), 4123-4128.
   Effect of the complement inhibitor eculizumab on thromboembolism in patients with paroxysmal nocturnal hemoglobinuria.
8. Hill A., Kelly R., Hillmen P. Blood 2013, 121(25), 4985-4996.
   Thrombosis in paroxysmal nocturnal hemoglobinuria.
9. Delvaeye M., Conway E., Blood 2009, 114(12), 2367-2374.
   Coagulation and innate immune responses: can we view them separately?
10. Weitz I., Razavi P., Rochanda L. et al. Thrombosis Res 2012, 130(3), 361-368.
    Eculizumab therapy results in rapid and sustained decreases in markers of thrombin generation and inflammation in patients with PNH independent of its effect on hemolysis and microparticle formation.
11. Hillmen P., Muus P., Roth A. et al. Br. J Haematol 2013, 162(1), 62-73.
    Long term safety and efficacy of sustained eculizumab treatment in patients with paroxysmal nocturnal haemoglobinuria.
12. Kelly R., Hill A., Arnold L., et al Blood 2011, 117 (25): 6786-6792.
    Long term treatment with eculizumab in paroxysmal nocturnal hemoglobinuria: sustained efficacy and improved survival.

2. Eculizumab dramatically improves the long term survival and quality of life of PNH patients.

Prior to eculizumab becoming available for clinical use, Hillmen et al carried out a detailed longitudinal study of quite a large cohort of PNH patients that had been followed up for over 30 years at Hammersmith Hospital in London, UK. That study showed that 30% of PNH patients were dead within 5 years of diagnosis, and by 10 years, 46% of patients were deceased[1]. The commonest cause of death was either directly or indirectly from thrombotic episodes. Many of these occurred despite either primary or secondary prophylaxis with Warfarin +/- aspirin.

Since eculizumab has been shown to be a highly effective therapy in PNH patients, no placebo-controlled study of eculizumab treatment in PNH patients will ever be conducted. To do so, would now be quite unethical and unacceptable. Hence any survival data must be based on historical comparative data involving selected cohorts of eculizumab-treated PNH patients. There are two such Eculizumab-treated cohorts where overall survival has been analysed.

The first of these is the UK cohort, the results of which were published initially in 2011[2] and again updated in 2012[3]. In the 2011 publication the results of eculizumab treatment in the first 79 patients were described. In the 2012 ASH Abstract, the results of eculizumab treatment in 153 UK patients were reported. Besides showing the more immediate short term benefits of eculizumab treatment, the survival outcomes in this cohort were also dramatically improved. Copied below is the survival curve from the 2011 Blood paper. This graphically demonstrates how eculizumab treatment of PNH patients increases their survival such that it **now matches an aged and sex matched normal population!!**



The second cohort study, based on an international group of PNH patients that were initially enrolled in to the TRIUMPH and SHEPHERD studies, was published in the British Journal of Haematology only 2 weeks ago[4]. Many of these 195 patients have now been on eculizumab treatment for upwards of 8 years. Over that prolonged period of the study, only 4 PNH patients have died whilst on eculizumab. This is a remarkable outcome for a large group of PNH patients treated at quite a number of different centres worldwide.

The overall survival curve from this latest British Journal of Haematology paper is copied below. As you can see this landmark survival analysis, performed at a median follow-up of 30 months, shows a remarkable overall survival for this PNH cohort.

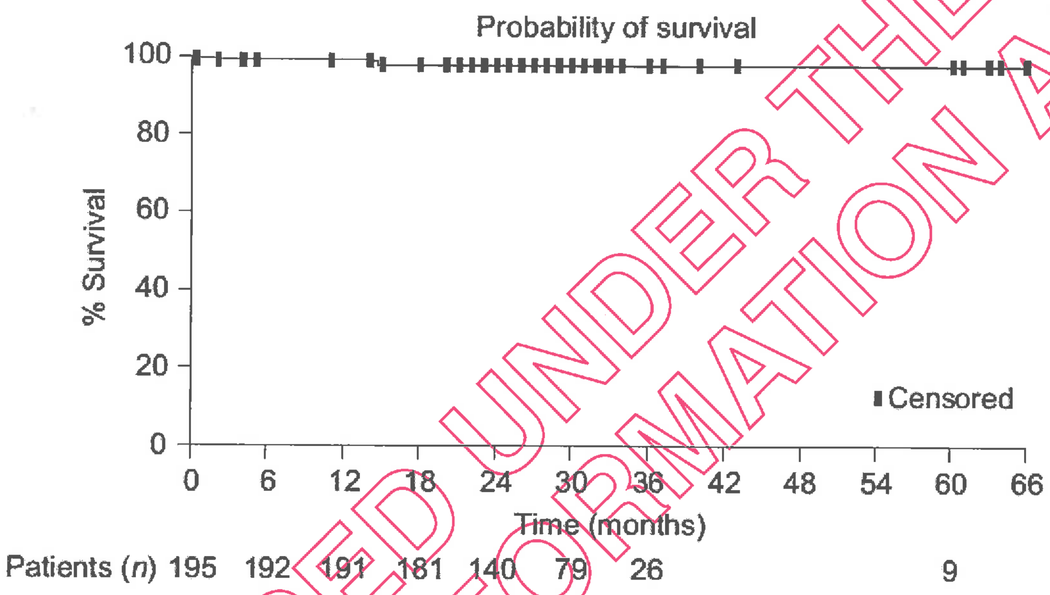


Fig 4. Long-term survival with eculizumab therapy.

Hence one must conclude that eculizumab is a highly effective therapy in the treatment of PNH, which dramatically alters the natural history of this disease, and markedly improves survival. **There are very few drugs in modern medicine that are so effective!!**

These benefits of eculizumab are thought to predominately originate from its ability to not so much switch off haemolysis, but rather to effectively prevent life-threatening thrombosis[5]. The above recent survival analyses have allowed statisticians to develop predictive survival models for PNH. These suggest that eculizumab treatment will result in prolongation of survival in PNH patients by in excess of 20 years. This again suggests that eculizumab treatment results in a huge health gain for any individual PNH patient.

Quality of Life (QoL) improvement

The SHEPHERD study[6], which involved a group of 97 transfusion-dependent PNH patients, formally assessed QoL using the EORTC QLQ-C30 instrument. An improvement in scores was seen across the fatigue scale, the scale for global health status (p<0.001), on all 5 scales for functioning (p<0.001) and on all 3 symptom scales (p<0.002). A summary table from that paper is included below:

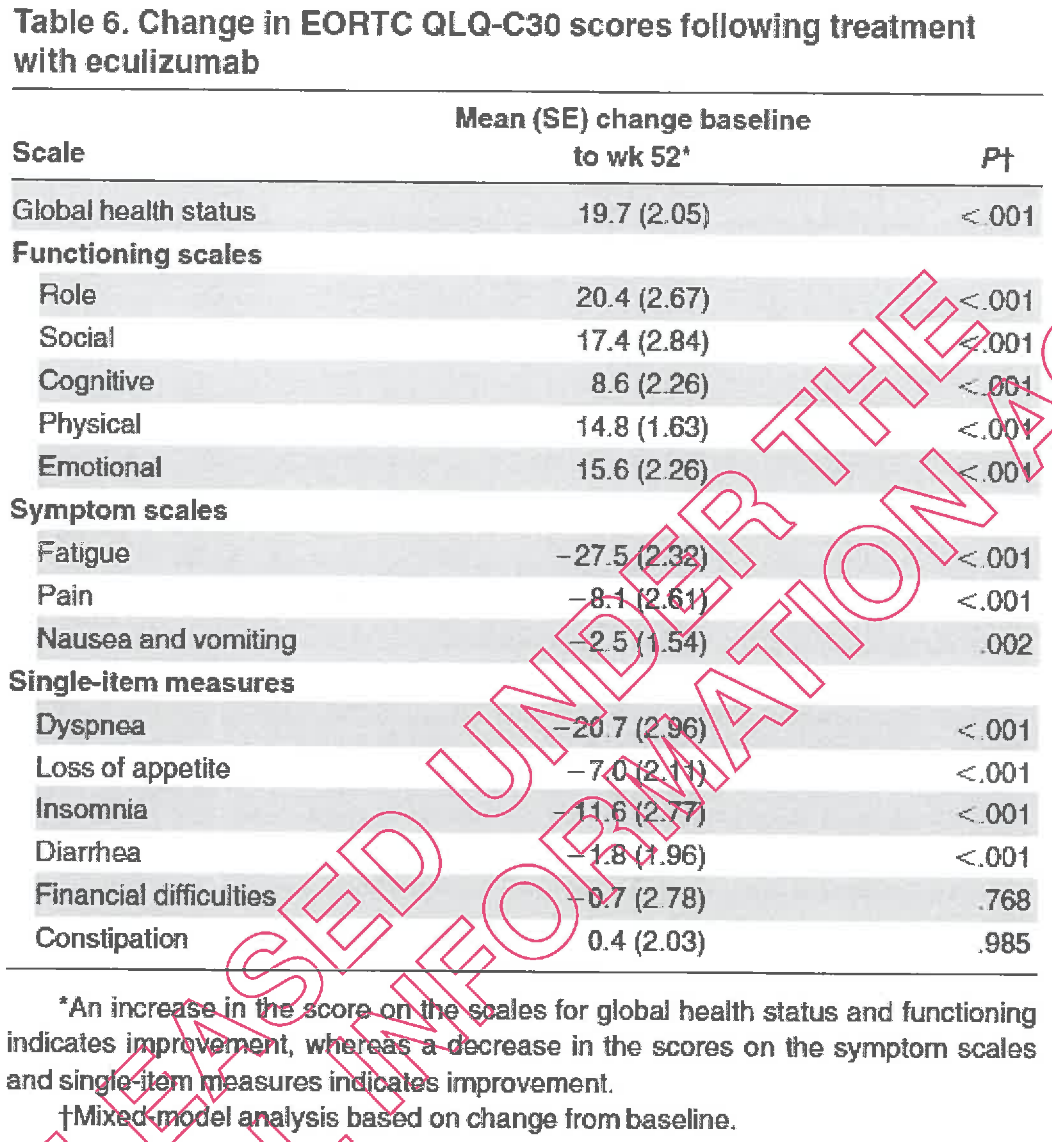


Table 6. Change in EORTC QLQ-C30 scores following treatment with eculizumab

Scale	Mean (SE) change baseline to wk 52*	P†
Global health status	19.7 (2.05)	<.001
Functioning scales		
Role	20.4 (2.67)	<.001
Social	17.4 (2.84)	<.001
Cognitive	8.6 (2.26)	<.001
Physical	14.8 (1.63)	<.001
Emotional	15.6 (2.26)	<.001
Symptom scales		
Fatigue	−27.5 (2.32)	<.001
Pain	−8.1 (2.61)	<.001
Nausea and vomiting	−2.5 (1.54)	.002
Single-item measures		
Dyspnea	−20.7 (2.96)	<.001
Loss of appetite	−7.0 (2.11)	<.001
Insomnia	−11.6 (2.77)	<.001
Diarrhea	−1.8 (1.96)	<.001
Financial difficulties	−0.7 (2.78)	.768
Constipation	0.4 (2.03)	.985

*An increase in the score on the scales for global health status and functioning indicates improvement, whereas a decrease in the scores on the symptom scales and single-item measures indicates improvement.

†Mixed-model analysis based on change from baseline.

Hence eculizumab treatment not only dramatically improves long term survival, but also results in a very prompt reduction in symptoms from PNH, which is directly translated in to an improved quality of life.

Eculizumab is a HIGHLY EFFECTIVE treatment for patients with severe, haemolytic paroxysmal nocturnal haemoglobinuria. **It should undoubtedly be funded and made available to NZ patients with severe, symptomatic disease.**

On Page 8 of the Consultation Document it is stated: "The high cost of a medicine is not necessarily a barrier to funding, provided that the expected benefits are reasonably proportional to the cost…". **Quite clearly the expected benefits from eculizumab treatment are so great, such that it is very reasonable to fund this medication even at a relatively high cost.**

References:

1. Hillmen P., Lewis S., Bessler M., et al., NEJM 1995, 333 (19), 1253-1258. Natural history of paroxysmal nocturnal hemoglobinuria.
2. Kelly R., Hill A., Arnold L., et al Blood 2011, 117 (25): 6786-6792. Long term treatment with eculizumab in paroxysmal nocturnal hemoglobinuria: sustained efficacy and improved survival.
3. Hill A., Kelly R. Hillmen P. et al, ASH 2012 Abstracts, Blood, 120, 3472 Eculizumab in Paroxysmal Nocturnal Haemoglobinuria (PNH): A report of all 153 patients treated in the UK.
4. Hillmen P., Muus P., Roth A. et al. Br. J Haematol 2013, 162(1), 62-73. Long term safety and efficacy of sustained eculizumab treatment in patients with paroxysmal nocturnal haemoglobinuria.
5. Hill A., Kelly R., Hillmen P. Blood 2013, 121(25), 4985-4996. Thrombosis in paroxysmal nocturnal hemoglobinuria.
6. Brodsky R., Young N., Antonioli E., et al Blood 2008 ,111 (4) 1840-1847. Multicenter phase 3 study of the complement inhibitor eculizumab for the treatment of patients with paroxysmal nocturnal hemoglobinuria.

3. PHARMAC have not consulted with either NZ or international haematologists who have experience in treating PNH patients with eculizumab, and therefore does not appreciate the dramatic benefits of eculizumab treatment.

PHARMAC received an application to fund eculizumab (Soliris) for the treatment of PNH from the supplier, Alexion Pharmaceuticals, in November 2011. Since that time, PHARMAC has sought advice from haematologists and others involved in the management and care of patients with PNH with regard to that application for funding.

In particular, advice was sought from the Haematology Sub-committee of PTAC, who discussed this matter formally at their meeting in August 2012. The outcome of those discussions has been detailed in previously published Minutes, and I note those recommendations were tabled at the Teleconference Meeting of PTAC held on 18 March 2013.

Aside from the fact that the initial recommendation was for a low priority for funding, none of the other recommendations from the Haematology Sub-committee have been acted on. In particular the concept of making eculizumab only available to those patients with more severe PNH has not been explored further. Likewise, the Haematology Sub-Committee has not been asked to re-evaluate their recommendations in the light of additional scientific publications about eculizumab in the treatment of PNH that have come to hand since August 2012 (See references in other sections above).

I must also point out that whilst the Haematology Sub-Committee of PTAC is made up of a number of eminent NZ Haematologists, NONE of them have had any experience in the treatment of PNH patients with eculizumab.

Alexion Pharmaceuticals has kindly made available supplies of eculizumab to 3 severely affected NZ PNH patients on compassionate grounds. The NZ haematologists who either currently or formerly care for [redacted] Those haematologists should have been consulted, to give first hand experience of how treatment with eculizumab has dramatically changed the lives of these 3 patients. This treatment is highly effective and dramatically improves quality of life. (See references in earlier sections above). Indeed all 3 of those NZ PNH patients may not currently be alive if it wasn't for compassionate access to this medication.

PHARMAC should also have consulted with international experts who have experience with eculizumab treatment in PNH. In particular, to hear from Professor Peter Hillmen, of Leeds, UK or from Professor Jeff Szer of Melbourne, Australia would help PHARMAC appreciate why eculizumab should be made available to NZ PNH patients.

I would argue strongly that PHARMAC should look at a further round of expert consultation, both nationally and internationally, before moving forward with their proposal to decline funding for eculizumab

4. **The number of NZ patients with severe, haemolytic PNH who really require treatment with eculizumab is relatively small. What's more, not all severe PNH patients need to remain on treatment with eculizumab long-term.**

On Page 4 of the Consultation Document it is stated "There are an estimated 60-70 patients with PNH in New Zealand and depending on the access criteria, a sub-group of 12-20 patients who might be considered for funded eculizumab treatment." The document then goes on doing all further calculations of cost based on treating 20 such patients. To me these calculations are both spurious and erroneous.

Although the internationally quoted prevalence of PNH is said to be less than 1 in 100,000[1], it is important to realize that there is considerable variation in the presentation, clinical manifestations and natural history of the disease amongst PNH sufferers. A working classification has been developed whereby three related but somewhat different presentations of the disease are recognized. In particular we now recognize (1) classic or haemolytic PNH, (2) PNH in the setting of another specified bone marrow disorder, for example PNH/aplastic anaemia, or PNH/refractory anaemia-myelodysplastic syndrome, and (3) subclinical PNH (PNHsc) in aplastic anaemia[2]. What is more, if studied by very sensitive techniques, even normal individuals can be found to have very low level PNH-like haematopoietic clones[3].

Hence, although PNH may in fact be much more common than first thought, it is important to realize that only patients with the more severe type of classical or haemolytic PNH should potentially be treated with eculizumab. Those patients have PNH haematopoietic clones that are readily detectable by conventional laboratory techniques, which are typically in excess of 10% of the total number of haematopoietic cells. It is for that reason that the Life Saving Drug Programme in Australia has chosen to include a minimum clone size of greater than 10% in their access criteria for federally funded eculizumab treatment[4].

Therefore, in order to ascertain how many NZ patients may potentially require treatment with eculizumab, it is important to ask:

a) Based on other countries where eculizumab is available, what is the current prevalence of those with classical haemolytic PNH with a large enough clone size to justify eculizumab treatment? And
b) How many such patients are we aware of currently in New Zealand?

In reference to a),
I have firstly consulted with Professor Peter Hillmen from Leeds, who heads up the nationwide, NHS-funded, UK programme of eculizumab treatment for patients with PNH. He is confident that all eculizumab-eligible PNH patients within the UK are now included in his programme. Hence, based on the UK prevalence, 2.5 to 3 patients per million population require treatment with eculizumab. Professor Hillmen therefore suggests that for New Zealand, some 10 to 12 patients will require eculizumab treatment – no more (personal communication).

In addition in Australia, where eculizumab has been available since December 2010, there are currently 57 patients on their eculizumab treatment programme, for a population of 22.6 million people. This is a rate of 2.52 patients per million population.
(This ties in perfectly with the figure quoted by Professor Hillmen from the UK). Based on the Australian prevalence figure, for a New Zealand population of 4.3 million people, we would expect 11 patients to require eculizumab treatment if the Australian access criteria were used.

In reference to b) above,
The NZ Branch of the Haematology Society of Australia and New Zealand (HSANZ) undertook a nationwide survey of all NZ haematology centres in 2011, looking to capture all known cases of diagnosed PNH. A total of 21 cases were identified, with a wide age range, the youngest patient being only 9 years old. Most of those patients had only mild disease, or had low-level PNH clones in the setting of either hypoplastic or aplastic bone marrows. Not all patients had had their PNH clone size accurately assessed. However based on that information, only 8 to 10 patients had severe haemolytic PNH such that they would warrant treatment with eculizumab. Although there have been some changes in that data since 2011, the PNH database has been kept up to date, such that the NZ haematology community is very confident that no additional PNH patients are about to suddenly "come out of the woodwork".

If we accept then that there are only 10 NZ PNH patients that require treatment with eculizumab, that puts a very different light on the pharmacoeconomics of funding eculizumab in NZ. Even if the purchase price for eculizumab was the quoted $600,000 per year, the total cost to PHARMAC would be only $6,000,000 annually; not the $12,000,000 quoted in the Consultation Document!

The long term requirement for eculizumab
On Page 3 of the Consultation Document it states: "…eculizumab is not a cure for PNH but it relieves the symptoms associated with PNH…….and it needs to be administered for the rest of the patient's life".

Whilst this statement is largely true, I think all international PNH experts would agree that we do not really know how the natural history of PNH may change in those patients who remain on eculizumab for a sustained period over many years. In other words, if eculizumab effectively prevents patients dying from the complications of PNH, what will happen to the PNH haematopoietic clone in the bone marrow over time??

Interestingly, what we are now beginning to see in some eculizumab-treated patients is that the PNH clone spontaneously begins to decline, and normal haematopoiesis is slowly re-established. In recent discussions with Professor Hillmen from the UK, he has highlighted how 2 patients in their UK eculizumab-treated cohort have had their PNH clone drop to such a low level that they have been **able to safely discontinue the drug!** There are another 6 UK patients with declining PNH clones that are being watched closely, and who may be able to come off eculizumab in the near future (personal communication).

Hence funding treatment with eculizumab may not be a life-long commitment for all PNH patients. Treatment of PNH patients with eculizumab has opened the door on the pathogenesis and biology of PNH a bit wider. Over time we may well find new therapies emerge such that eculizumab provides a bridge to curing the disease long-term.

Finally I must point out that, although eculizumab is currently regarded as a very expensive medicine, it will not always remain so. The world-wide patent that Alexion Pharmaceuticals has on eculizumab at present is likely to expire in the next 7 to 10 years. After that, generic forms of eculizumab will almost certainly become available, at a very much cheaper price. So whilst the commitment to fund eculizumab in NZ at a potential annual cost of $6,000,000 sounds a lot of money going forward, it would not need to be sustained indefinitely. That cost is likely to drop quite dramatically as soon as the drug comes of patent in some 7 to 10 years. It would be a shame if a number of NZ PNH patients died without eculizumab treatment during that period.

References:

1. Luzatto L. and Notaro R. in Postgraduate Haematology, 5th Edition (2005), Blackwell Publishing, Chapter 11, page 169.
2. Parker C., Omine M., et al Blood 2005, 106(12): 3699-3709. The Diagnosis and Management of paroxysmal nocturnal haemoglobinuria.
3. Araten D., Nafa K. et al Proc Natl Acad Sci U.S.A. 1999; 96:5209-5214. Clonal populations of hematopoietic cells with paroxysmal nocturnal hemoglobinuria genotype and phenotype are present in normal individuals.
4. Guidelines for the treatment of Paroxysmal Nocturnal Haemoglobinuria (PNH) through the Life Saving Drugs Program.
   Available at www.health.gov.au/lsdp December 2010.

5. **Eculizumab could just be made available to the small number of NZ PNH patients with the more severe, haemolytic type of PNH using clearly defined but restrictive NZ access criteria.**

If PHARMAC has concerns about the very high cost of eculizumab, then one approach to dealing with that is to only make eculizumab available to those NZ patients with the most severe forms of haemolytic PNH.

In that regard, I note the Haematology Sub-Committee of PTAC did suggest, at their meeting in August 2012, that perhaps PHARMAC should consider funding eculizumab with very restrictive access criteria. They suggested that PNH patients in NZ had to fulfil the following criteria:

1. To have developed a radiologically proven thrombosis, despite adequate anti-coagulation, or
2. Have a clone size of >50%, have systemic symptoms (for example severe abdominal pain, fatigue or shortness of breath) and have evidence of active haemolysis. (See PTAC Minutes, 18 March 2013).

One of the issues with the above suggestion would be that perhaps a clone size of >50% is a bit too restrictive, and secondly, that assessment of fatigue, and to some extent shortness of breath are subjective symptoms and may be difficult to judge.

Personally I would rather prefer adopting the Australian access criteria[1], but with some modification of clone size and transfusion requirements to make them a bit more restrictive. The Australian criteria are very robust, and have been in use for close to 3 years now. They have very little subjectivity in terms of patient assessment. They also mandate meningococcal vaccination prior to commencing eculizumab, and require a regular 6-monthly clinical review of every patient, which includes a re-assessment of clone size.

I would suggest making 2 changes to the Australian access criteria for use in NZ:
1. Increasing the clone size, somewhat empirically from 10% to 20%. It would be very important that clone size was assessed formally, using standardized criteria, preferably in one single laboratory in NZ.
2. Increasing the RBC transfusion requirement to more than 6 units of blood in 12 months, rather than 4 units.

Although I have not assessed these changes against the NZ patients currently enrolled on the NZ PNH database, I would be very happy to do so, recognizing that most patients would need to have their clone size re-assessed. I suspect however that these slightly more restrictive criteria would reduce the number of NZ patients that would be potentially eligible for eculizumab treatment, perhaps bringing the patient number down from 10 patients to around 8 patients. That could potentially reduce the budgetary impact of funding this medication, whilst still making it available to the most severely affected patients.

References:

1. Guidelines for the treatment of Paroxysmal Nocturnal Haemoglobinuria (PNH) through the Life Saving Drugs Program.
   Available at www.health.gov.au/lsdp   December 2010.

6. The potential cost of eculizumab to PHARMAC is much less than has been quoted.

On Page 4 of the Consultation Document it is stated "The pharmaceutical cost of eculizumab is estimated to exceed NZ$600,000 per patient per year; therefore funding eculizumab for PNH in New Zealand would cost approximately $12,000,000 (20 patients) per year. This cost would be likely to increase as more patients meet the access criteria and qualify for treatment each year."

I have had discussions with personnel at Alexion Pharmaceuticals about the potential cost of supplying eculizumab to NZ patients with severe PNH. Like any other pharmaceutical company negotiating a deal with PHARMAC to get their drug funded, Alexion are more than willing to supply the drug with a significant confidential price rebate. Price **discounts well in excess of 50%** have been mentioned to me in confidence. Obviously, owing to the commercial sensitivity of this, Alexion are not prepared to put anything in writing for me. However Alexion Pharmaceuticals are a company with a well respected international reputation, whereby I do believe they would negotiate in good faith to reach a fair and reasonable price. In previous discussions with PHARMAC, Alexion believe that PHARMAC have "walked away", because they were not genuinely interested in having eculizumab funded in NZ in the first place.

In regard to the concern that "costs would be likely to increase as more patients meet the access criteria….", Alexion Pharmaceuticals are very happy to discuss that issue. They are quite comfortable about entering into a risk-sharing proposal, if there are concerns about potential patient numbers increasing. Indeed, they have already done that in several other countries where eculizumab is available, and would be quite happy to do that for New Zealand. Unfortunately they cannot progress such a negotiation without PHARMAC being involved.

I would therefore request that PHARMAC meaningfully re-commence negotiations with Alexion Pharmaceuticals, before moving forward with their proposal to decline funding for eculizumab. PHARMAC might be pleasantly surprised by the level of co-operation and goodwill they receive.

7. **PHARMAC does not have a suitable process for the transparent and fair evaluation of high cost medicines used for the treatment of rare diseases. PHARMAC needs to address this, and if necessary should evaluate eculizumab via such revised criteria.**

In my discussions with PNH patient groups and health funders, I have been repeatedly reminded that because PNH is a rare disease, it may well not be appropriate to use PHARMAC's usual cost-utility analysis approach to make an informed decision about whether or not to fund eculizumab[1].

Before developing a Cost Utility Analysis (CUA) based on quality-adjusted life years (QALYs), reviewing clinical evidence to establish the extent of net clinical benefit is required. In that respect, analysing high level evidence based on randomised trials is preferred[1]. In the case of PNH however, where patient numbers are small, and a highly effective treatment is potentially available, no randomised trial of sufficient size is ever likely to be done. Indeed to do a placebo-controlled trial of eculizumab treatment in PNH **would now be considered unethical**. Hence, trying to get a strong case for funding eculizumab based on a compelling CUA is very difficult, as it is for the treatment of any other rare disease in NZ.

In recognition of that, it is interesting to note that in recent weeks the New Zealand Organization for Rare Disorders (NZORD) has become much more active, and has been holding a series of public meetings throughout NZ. NZORD feel quite strongly that the PHARMAC proposal to decline the funding of eculizumab for patients with PNH is fundamentally wrong. NZORD believe PHARMAC's current approach to assessing orphan drugs for rare diseases in NZ is flawed, and needs to be changed urgently. I would certainly support that view, in light of the arguments detailed above.

PHARMAC owe it to the small number of NZ PNH patients with severe disease, who have much to gain from eculizumab treatment, to re-assess the net health benefit from this drug. As I have presented in previous sections above, the potential gain in absolute and quality-adjusted life expectancy from eculizumab treatment of severe haemolytic PNH is HUGE. PHARMAC needs to be look at this critically once again, particularly in the light of recent publications[2].

References:
1. Grocott R., Metcalfe S., Alexander P., et al. NZMJ 2013, 126, 1378, 60-72.
   Assessing the value for money of pharmaceuticals in New Zealand: PHARMAC's approach to cost-utility analysis.
2. Hillmen P., Muus P., Roth A. et al. Br. J Haematol 2013, 162(1), 62-73.
   Long term safety and efficacy of sustained eculizumab treatment in patients with paroxysmal nocturnal haemoglobinuria.

Personal Statement and Declaration

In writing the above submission, I wish to share with you my motivation for taking on an advocacy role for PNH patients in NZ to obtain funded access to eculizumab treatment.

I have been practicing as a specialist haematologist in NZ for over 20 years. During that time I have fulfilled a number of leadership roles amongst my colleagues, including serving as the NZ Councillor to the HSANZ from 2005 to 2011.

During my time as a haematologist, nothing moved me more than when I met [redacted], a [redacted]-year-old PNH patient in [redacted] some years ago. [redacted] had been diagnosed with PNH when [redacted] first presented when [redacted] with a [redacted], which had developed as a result of a [redacted]. When [redacted], she developed a [redacted], and it was then that the diagnosis was made. Ever since then [redacted] had been on treatment with Warfarin and Aspirin. [redacted] PNH had turned [redacted] life in to a misery – [redacted] was a young person who was missing out on [redacted] most precious years, and had a very bleak future.

Somewhat surprisingly, after [redacted] mother directly approached and pleaded with Alexion Pharmaceuticals, a compassionate supply of eculizumab was made available for [redacted]. I remember vividly just a few weeks after starting eculizumab treatment, meeting [redacted] again in the outpatient clinic. The change was ABSOLUTELY DRAMATIC. [redacted] quality of life had turned around completely, and at last [redacted] had something to live for…

Eculizumab is a marvellous treatment for patients with severe haemolytic PNH, and it MUST be made available to more NZ PNH patients like [redacted]. I know it is an expensive medication, but somehow we must get this funded for PNH sufferers who have so much to gain through this treatment. I really ask for your help to make this possible.

Finally, I want to put on record, that I personally have nothing to gain from having eculizumab funded in NZ.

- I do not have PNH myself, nor do any of my family or friends.
- I do not have shares or any pecuniary interest in Alexion Pharmaceuticals.
- I have not been paid any fee or retainer by Alexion Pharmaceuticals.
- I will not receive any payment or any other reward from Alexion Pharmaceuticals, or any other third party, if eculizumab is eventually funded in NZ.

I believe in this campaign for eculizumab to be funded because I have seen first hand what a highly effective treatment it is; I have seen how it can really change lives for good; and I believe every NZ sufferer of severe haemolytic PNH deserves to receive this life-saving medicine.

Please PHARMAC, look at it again. Please DO NOT proceed with your proposal to decline to fund this application.

Dr. Humphrey Pullon, MBChB, FRACP, FRCPA
Consultant Haematologist
Waikato Hospital, Hamilton.

To: eculizumabfeedback[eculizumabfeedback@Pharmac.govt.nz];
Subject: Eculizumab
Sent: Tue 7/30/2013 10:04:15 **
From: Andrew Butler

Thank you for the opportunity to feed back on the PHARMAC proposal.

I support the proposal for restricted access for severely affected patients. The reasons for this have already been meticulously documented in the submission by my colleague, Dr Humphrey Pullon, and do not need repeating.

Best wishes,

Andrew

Dr Andrew Butler
Haematologist | Canterbury Health Labs [redacted]
P [redacted] | F [redacted] | E [redacted]

Sue Anne Yee
Therapeutic Group Manager
PHARMAC

14 June 2013

Dear Sue Anne

Thank you for the opportunity to comment on the recently announced proposal to decline funding for eculizumab for patients with paroxysmal nocturnal haemoglobinuria (PNH). The following comments are my personal views as a consultant haematologist who manages patients with this condition. They should not be considered to represent the views of the New Zealand branch of the Haematology Society of Australia and New Zealand, of which I am currently the New Zealand councillor, nor of the Haematology Work Group, which I currently chair. I have encouraged members of those bodies to make individual submissions, since I do not believe there is a consensus amongst us on this issue that would allow a joint submission on behalf of these groups.

My comments are from the perspective of a clinician with some experience in diagnosing and managing PNH as well as numerous other conditions for which effective but unfunded treatment options exist. The points I would like to make are:

1. I acknowledge the burden that this disease represents for those patients unfortunate enough to suffer from it and I have considerable sympathy with their desire to gain funded access to this drug.
2. As a physician I have a duty to offer my patients the best possible care available and I would like to be able to offer this therapy to appropriate patients with this disease, because the evidence is clear that eculizumab is very effective in reducing haemolysis, transfusion requirements and some of the complications of this disease as well as improving quality of life for sufferers. It may also reduce the risk of dying from complications of this disease, although the data on survival are less clear, relying on selected retrospective analyses.
3. However, because of the extraordinary cost of this treatment, I believe that a broader perspective is necessary in considering this issue and that we also have a duty as clinicians to consider the financial implications of the treatment decisions we make because they inevitably impact on other patients, given that resources are finite.
4. The fact that this drug has been funded in other countries should not alter the fact that New Zealand has to make its own decision about this with reference to the health needs of our own population and the resources available in this country. Funding decisions made in other developed countries should not automatically be adopted here, in my opinion.
5. The number of patients with this disease in New Zealand appears to the source of some disagreement, aired in the initial media response to this proposal. The estimated prevalence frequently quoted in the literature (Rosse WF, Epidemiology of PNH, Lancet 1996, 348:560) is 1-5 cases per million, which would predict for approximately 4-20 cases in New Zealand, although other estimates of prevalence are considerably higher. My understanding is that there are about 18-20 patients recognised to have this disease in New Zealand and that about half of them might meet the proposed treatment criteria. However, I acknowledge that this understanding is based on informal communication with colleagues and representatives of Alexion Pharmaceuticals as well as my understanding of data from the international PNH registry, funded by Alexion Pharmaceuticals. The number of eligible patients is critically important when considering the total cost of this drug to the country, because of the extreme cost per patient. However, funding it for even a very small number of patients could have considerable financial implications, with clinical implications for a potentially much greater number of patients deprived of effective treatments because of the finite resources available to fund healthcare in this country.
6. Although this particular condition is very rare, "rare diseases" as a broad group are relatively common. EURORDIS, the European Rare Disease organisation estimates that 6-8% of the European population suffer from a "rare disease" (defined as a disease with a prevalence of <1:2000). Although funding eculizumab for PNH would represent a small proportion of the pharmaceutical budget because of the rarity of the disease, such funding potentially sets a precedent. Other companies seeking funding for rare disease treatments are likely to view the price achieved as a benchmark. Collectively, funding expensive rare disease treatments could have enormous financial implications if the cost of treating each disease is similar to that of using eculizumab for PNH.
7. The clinical severity of PNH is very variable, with some patients suffering severe thrombotic complications, transfusion dependence, and disabling symptoms, while others have few or intermittent symptoms and a relatively low or no transfusion requirement. The proposed symptomatic criteria

(severe abdominal pain, fatigue, shortness of breath) are subjective, relatively non specific and open to considerable variability in interpretation. It seems hard to justify spending over half a million dollars per year to improve fatigue in a single person, which would appear to be possible under the proposed criteria. It may be appropriate to consider a different threshold for funding a small number of the most severely affected patients (particularly those with severe thrombotic complications) rather than simply adopting the arguably quite liberal criteria used in some countries for such extraordinarily expensive therapy.

8. While other treatment options are limited, there are some therapeutic strategies of benefit in some patients.
    a. Although the subject of some debate, in my experience corticosteroids have some activity and can be effective in some patients, both in the acute setting, and in lower doses longer term. While not as reliably effective as, and potentially more toxic than eculizumab, this class of drugs is a reasonable option in some clinical circumstances, in my opinion.
    b. Transfusion therapy, although not ideal, allows many patients to lead a relatively normal life. There are numerous patients with other haematological disorders who are transfusion-dependent and who are unable to access drugs considerably cheaper than eculizumab (erythropoietin, azacytidine, lenalidomide, etc) that have the potential to reduce their transfusion requirements, improve their quality of life and extend their survival.
    c. Allogeneic transplantation is the only curative strategy for this disease. Although there are risks with this therapy, it has been used successfully in this condition in New Zealand and the safety of this form of treatment for other conditions is improving. Patient selection is critical and the outcomes are particularly good in PNH patients transplanted for haemolysis without complicating thrombosis or aplastic anaemia (deLatour et al, Haematologica, 2012;97:1666-73). Although this form of therapy is also expensive, the average cost of an allogeneic stem cell transplant is a fraction of the annual cost of eculizumab therapy for one patient.

Therefore, despite my sincere concern for the patients who suffer from this disease and my desire to give them this treatment, I wish to express my strong support for the proposal to decline funding for this drug, based on its extreme high cost. Although I do not have any information about the actual price that might be achievable through negotiation, unless this were to be a small fraction of the suggested price of over $500,000 per patient per year, it is my opinion that much greater health benefits could be achieved for the New Zealand population by spending this money on other initiatives. Nevertheless, as a compromise, it may be appropriate to explore different thresholds for funding this drug for a very small number of patients with the most severe clinical manifestations of this disease.

Yours sincerely,

Dr Bart Baker (FRACP, FRCPA)
Consultant Haematologist
Regional Cancer Treatment Service
Palmerston North Hospital

From: Helen McDermott [redacted]
Sent: Thursday, 1 August 2013 12:03 a.m.
To: eculizumabfeedback
Subject: PNH patients
Attachments: PNH submission.docx

Dear Sue Anne,

Please find attached my submission to be considered for the Eculizumab feedback,

Kind regards,
Helen McDermott

Canterbury
District Health Board
Te Poari Hauora ō Waitaha

31 July 2013

Sue Anne Yee
Therapeutic Group Manager
PHARMAC
PO Box 10254
Wellington 6143

Dear Sue Anne,

I am a research nurse who has been involved in the haematology department for over 15 years. The PNH registry was opened in Christchurch in 2006 and then in the other main centres in New Zealand. This gave us the ability to compile data on PNH patients in New Zealand.

This data was then combined into the international PNH registry to observe the natural history and management of those with PNH.

This registry has highlighted disparities between patients in countries with access to Eculizumab and those with no access. As the years have gone on most other countries now have Eculizumab however New Zealand does not.

The registry now provides data on those patients around the world whose lives have had a dramatic improvement with the provision of Eculizumab, while New Zealand PNH patients provide data on the progression of their disease without treatment and the impact on their lives through the Quality of Life forms.

We have 3 patients in New Zealand on Eculizumab through the compassionate use programme and it makes for inspiring reading as these 3 all were in life threatening situations in intensive care. Now they are all working and contributing in their own way to our communities all with a future that is as certain as any of us.

It is difficult to watch the gradual decline of people struggling each day with their condition knowing a proven treatment is available to other sufferers around the world which is being denied to our patients. There is no other treatment besides trying to manage the symptoms of their disease. Blood transfusions, warfarin to try to prevent blood clots and a listening ear is all we can do. The amount of blood transfusions required increases as does the frequency of hospital visits from pain, especially abdominal, from clotting episodes and renal impairment.

These few people who suffer terribly from this chronic and progressive disease deserve the right to have access to a treatment that is proven to be effective.

I acknowledge that PHARMAC have reviewed the data closely however I am puzzled at the numbers that PHARMAC have come up with, they don't correlate with the numbers I and others have spent time gathering over the years. I urge PHARMAC to continue discussions with the aim of finding a workable criteria for access to Eculizumab and a funding solution.

Kind regards,

Helen McDermott
Haematology Department
Christchurch

[redacted]

[illegible] 12 AUG 2013

Department of Haematology
Dunedin Hospital
[redacted]

4 August 2013

Ms. Sue Anne Yee
Therapeutic Group Manager
PHARMAC
PO Box 10 254
WELLINGTON 6143

Dear Sue Anne,

Re: Feedback submission on PHARMAC's proposal to decline a funding application for Eculizumab

I have two patients with PNH who are on compassionate supply of Eculizumab. I have data to demonstrate that my two patients had had lifethreatening complications from their disease. If it was not for Eculizumab both patients could have had died of complications from their PNH.

PNH is a rare disease and during my haematology training in Adelaide, Australia I did not have a personal experience in the treatment of this condition. I met [redacted] after arriving in NZ in August 2006. [redacted] was under the care of Dr Musuka and I took over her care after his departure.

[redacted]

In 2007 [redacted] PNH was getting out of control, during this year [redacted] had 15 admissions and spent 72 days in hospital. [redacted]

By August 2008 [redacted] was on [redacted] 8th admission this time with [redacted]

During this admission because of the severity of [redacted] PNH we contacted the [redacted] Bone Marrow Transplant Committee to consider [redacted] for myeloablative unrelated allogeneic stem cell transplantation.

The Bone Marrow Transplant Committee suggested I contacted Prof P Hillmen, which I did and Prof P Hillmen's advice was that this procedure was contraindicated on [redacted] He advised that we should approach Alexion Pharmaceutical to request compassionate supply of eculizumab, which I did and fortunately it was approved.

Since eculizumab was commenced [redacted] has had no further life threatening [redacted], with [redacted] last admission in April 2012.

[redacted] has not been admitted to hospital since then, moreover, [redacted] is working full time [redacted] On top of this [redacted] had frequent admissions for pain control [redacted]

In the long term as you can see eculizumab has been a lifesaving medication for [redacted]. [redacted] is no longer suffering from a debilitating abdominal pain and [redacted] is no longer at risk of life threatening [redacted].

She is living a normal life, working full time and contributing with her taxes to our community instead of living on a health benefit.

I would like to expand on the health benefits [redacted] my other patient has had but suffice to say that [redacted] approval was based on the lifesaving properties of eculizumab without which [redacted] could have died in a few days from a [redacted] [redacted] has not had admission to hospital since the administration of eculizumab in February 2011 and [redacted] is also working part time.

I have attended a few meetings supported by Alexion to learn more about eculizumab.

I have no financial interest with the company and I have not been paid any fee or other pecuniary interest to speak favourably about the drug.

The Medical Records of my two patients can be seen as we keep them on i-soft. Also the original Medical Records can be reviewed if needed.

I have taken over the care of haemophilia patients and with a particular patient the cost for [redacted] haemophilia treatment from the period of the 1/7/11 to 30/6/13 was 438,500. I presume that for the next few years this cost will be more or less similar depending on the numbers of unpredictable bleeds my patient could have.

Severe haemophilia is not a rare condition in comparison to PNH patients in New Zealand. Patients with mild and moderate haemophilia do need to be treated now and again throughout their life span. However, patients with PNH need to be treated when they have a life threatening condition, i.e. when they have severe disease.

If medical care in New Zealand is fair, which I believe it is then patients with severe PNH must be treated as patients with severe haemophilia are.

Therefore, I strongly support and plead that funding of eculizumab is made available by PHARMAC for patients with life threatening PNH in New Zealand.

Kind regards

Yours sincerely

Dr Hilda Mangos. MBChB, FRACP, FRCPA
Consultant Haematologist
Dunedin Hospital
Dunedin

To: eculizumabfeedback[eculizumabfeedback@Pharmac.govt.nz];
Flag Status: 0x00000000
Subject: Feedback on eculizumab
From: Hugh Goodman
Sent: Mon 5/27/2013 12:05:40 **

Dear Sue Anne

Thanks for the well-considered documentation around this difficult decision. I would like to make some general comments, then specifically on "how much is too much" and finally PHARMAC's apparent passiveness in price negotiations in this instance.

1. In general I support the conclusions drawn by CaTSoP, PTAC and PHARMAC staff. I believe that the drug is clearly of major benefit to a small number of patients in NZ, certainly prolonging life expectancy, improving quality of life and reducing other Vote Health expenditure. However, despite this, I agree that it is difficult to see how, **at the list price**, it represents value for money.

2. The question, therefore, is "how much is too much". I suggest 2 current benchmarks, being imatinib (and its kin) for CML and treatment of haemophilia. The former set the bar at $60-80k/yr/pt for a life-transforming therapy but, arguably, one that had an existing therapy in the form of allogeneic BMT. Haemophilia, although outside your funding remit, has a wide range of cost / pt but a severe (as in severely deficient factor 8) adult would usually consume ~$150-200k of F8 in prophylaxis and this might double if the joints are bad. Patients with inhibitors cost far more than this. One could argue, therefore, that the current standard for life-transforming therapies is an ongoing cost of $100-200k/pt/year. If one factors in the novelty and small numbers (which affects the returns to the supplier as well as your costs), I believe that a price of <$300k/pt/year would be reasonable in this case.

3. Lastly, I have substantial concern around PHARMAC's apparent approach to the commercial negotiation process. The documentation implies that you have not entered negotiations with the supplier. I know very well that the actual price, via "confidential rebates", for many other 'big ticket' drugs is far below the list price and although I have placed on record previously my dislike of this lack of transparency it does give suppliers scope to bring the price down substantially. To put this more simply, "have you negotiated with the supplier on price and if not, why not?".

If the price could be brought down to perhaps $300k/year/pt I would endorse its listing given there are probably only ~10pts that really need it.

Regards

Hugh Goodman

Dr Hugh Goodman

Haematologist | Waikato Hospital | [redacted]

[redacted]

To: eculizumabfeedback[eculizumabfeedback@Pharmac.govt.nz];
Flag Status: 0x00000000
Subject: eculizumab feedback
From: Paul Baines
Sent: Thur 5/23/2013 1:50:54 ..

Northland DHB have considered the ‘Proposal to decline a funding application for eculizumab’, and based on the analysis as presented within the proposal, Northland DHB support the decision to decline funding

Regards

Paul Baines

Primary Care Portfolio Manager

Northland DHB

[redacted]

[redacted]

[redacted]

[redacted]

[redacted]

Canterbury
District Health Board
Te Poari Hauora ō Waitaha

033872 31 MAY 2013

27 May 2013

Ms Sue Anne Yee
Therapeutic Group Manager
PHARMAC
P O Box 10 254
WELLINGTON 6143

Dear Sue Anne

Re: Pharmac Proposal to decline a funding application for eculizumab

I was sorry to learn that Pharmac propose to decline funding for eculizumab for any patient with paroxysmal haemoglobinuria.

I am responsible for the care of one of the very small number of patients with severe PNH in New Zealand. Therefore I must, as this patient's doctor and also in the role of [redacted] advocate, point out that Eculizumab would be a highly effective treatment for [redacted].

My patient is in [redacted] mid [redacted]s, [redacted] Eventually some or all of the many complications of PNH will occur and [redacted] life will be shortened dramatically.

On the other hand we know that Eculizumab is a highly effective treatment for patients with severe PNH. In particular eculizumab

- causes a marked reduction in red blood cell haemolysis
- markedly reduces the requirement for red blood cell transfusion, and in many cases removes this requirement completely
- markedly reduces the depletion of nitric oxide, which results from the presence of free haemoglobin in the circulation. As a result of this, the severe fatigue associated with PNH improves, and disabling abdominal pain, caused by muscle spasms and cramping resolves.
- markedly reduces the risk of developing thrombotic complications. This is especially important, since major thrombosis and the complications thereof is the major cause of death in PNH patients.
- markedly increases quality of life – as judged by formal QoL studies
- **markedly improves survival**
  Studies from the Leeds group in the UK have shown the survival of an eculizumab-treated PNH cohort of patients is now equivalent to age and sex matched controls. Some studies suggest a prolongation of survival in excess of 20 years!!

There is now overwhelming evidence of the effectiveness of eculizumab in the treatment of patients with PNH. Instead of PNH patients suffering from a progressive, debilitating disease, treatment with eculizumab allows them to return to having full and active lives, with the prospect of having a much longer survival.

Eculizumab is desperately expensive, that I know. Part of the problem is the cost being asked by the manufacturer, part of the problem is that we (Pharmac) have lots of other things to spend our money on. Please consider recommencing discussions with Alexion Pharmaceuticals so that those very few patients with severe PNH can have access to treatment with this highly effective drug.

Yours sincerely

P Ganly

Electronically checked and signed by:
Peter Ganly
CONSULTANT HAEMATOLOGIST

Department of Haematology
Canterbury Health Laboratories, P O Box 151, Christchurch, NZ
[redacted]
[1]HAEMATOLOGISTS: Dr A Butler, Dr P Ganly, Dr S Gibbons, Dr S MacPherson, Dr M Smith, Dr R L Spearing

From: Ruth Spearing [redacted]
Sent: Sunday, 28 July 2013 5:04 p.m.
To: eculizumabfeedback
Subject: Submission on the use of eclizumab in PNH patients

Thank you for the chance to comment on this subject.

I recognise that this is a very expensive drug because of the very small potential market and the high costs there have been in developing it. However within this is a group of patients there are those who have very severe disease and are likely to die as a result. My understanding is that there have been 2 deaths in the last 12 months.

It is therefore vital that Pharmac look at ways of identifying the most severely affected patients, if it believes it cannot use the same criteria as the Australians for financial reasons. Under the Australian criteria there would be 12 patients who would be eligible. If the criteria was made more strict eg 60% clone and a history of thrombosis this would reduce the number down to 6. – this would al least ensure the most severely affected patients are treated. This approach does carry the risk that the first episode of thrombosis may be fatal but it will at least allow the most severely affected patients to be treated.

Not to allow treatment to the most severely affected patients who have a significant chance of dying from their condition is morally unacceptable. In a country where a single DHB spends $63 million on treatment of alcohol related harm, it is inappropriate to withhold treatment from this small group of patients with very severe disease who have a significant chance of dying from it.

Having seen first hand the dramatic improvement in quality of life with treatment with the use of the drug (given on a compassionate basis) I would urge Pharmac to reconsider it position with regard to this drug.

Yours sincerely

Ruth Spearing

Dr Ruth Spearing,
Haematologist, Christchurch Hospital,
[redacted]

From: [redacted]
Sent: Wednesday, 31 July 2013 12:33 p.m.
To: OPP Review
Subject: FW: Submission to PHARMAC's Proposal to decline a funding application for Soliris (eculizumab)

[redacted]

Please email *BOTH*

Subject: **Submission to PHARMAC's Proposal to decline a funding application for Soliris (eculizumab)**
Email: eculizumabfeedback@pharmac.govt.nz

AND

Subject: Submission in response to PHARMAC's consultation on Decision Critera.
Email: opp@pharmac.govt.nz

To Whom it May Concern;

I support PNH patients in New Zealand in their fight to gain access to the life-saving treatment Soliris.

I DO NOT support PHARMAC's proposal to decline funding for the Soliris treatment.

I support the PNH Support Association's proposition for Equity and Fairness which states:

i. **PHARMAC must return to the negotiating table** with the supplier of the Soliris treatment

ii. **PHARMAC must negotiate in good faith** toward funding Soliris for a minimum of 8 NZ PNH patients

iii. **PHARMAC must establish fair assessment criteria**, based on expert advice from the international haematological community, to assess patient need for the Soliris treatment

iv. **PHARMAC must amend its Operating Policies and Procedures** to acknowledge the right of rare disease patients to access life restoring and life-saving treatments as in the specific example of the Soliris treatment

(please include your optional personal statements here)My Sister needs to have the choice of living back in New Zealand and have the support of her family

Yours faithfully

[redacted]

---------- Forwarded message ----------
From: [redacted] [redacted]
Date: 11 June 2013 16:49

Subject: Submission to PHARMAC's Proposal to decline a funding application for Soliris (eculizumab)
To: eculizumabfeedback@pharmac.govt.nz

To whom it may Concern,

I am a New Zealander and I suffer from PNH. [redacted]

[redacted] has recently offered me treatment with Soliris due to the continuing seriousness of my condition. But should Soliris continue not to be funded in New Zealand, I, my New Zealand-born partner and any future children we may have, will effectively be exiled from our homeland, as once I embark on the drug treatment, I must remain on it or face life threatening consequences.

I will therefore be forced to choose my physical health over my emotional health which would derive from being able to return to New Zealand to live, and be surrounded by family, in the country in which I grew up and spent much of my adult life.

This is an unacceptable position in which to be placed. I have delayed starting treatment with Soliris (possibly to my detriment) because I am waiting for the outcome of the current PHARMAC consultation regarding this issue. If Soliris is not funded in New Zealand, this will affect whether I commence this treatment which I anticipate will provide me with an increased quality of life and also an increased life expectancy.

[redacted]

[redacted]

Most importantly I am of course aware that Soliris is not currently funded in New Zealand. As I am sure you will be aware, once treatment with Soliris is commenced, it cannot be stopped without elevating the inherent risks of PNH including the increased risk of thrombosis.

Therefore the fact that Soliris treatment is not funded in New Zealand is perhaps the most significant factor in my decision whether to commence this recommended treatment. I always intended to return to New Zealand to live. Should Soliris continue not to be funded in New Zealand, I will be prevented from returning to my homeland.

The personal consequences of not being able to live in New Zealand in the future are too numerous to list here. [redacted] I also have a large extended family living in New Zealand who I will be prevented from having any real future contact with, not to mention close friends. My boyfriend with whom I live is also a New Zealander and should I be prevented from returning to the land of my birth, so too will he if we wish to be able to reside in the same country.

Additionally, any children I am lucky enough to be able to have (I understand that pregnancy is only possible for PNH sufferers who are undergoing Soliris treatment) will therefore be prevented from living in New Zealand, knowing any of their grandparents or other family members.

Apart from the important personal consequences this presents, from a professional perspective, I had also hoped to utilise the wealth of knowledge and experience I have gained [redacted], on my return to New Zealand.

I urge you to reconsider your stance on the funding of this drug. I can't imagine how stressful it must be for the other New Zealanders with PNH and their families who are having to deal with this illness, knowing that there is a drug which could increase their quality of life and also their life expectancy but not having access to it.

I support PNH patients in New Zealand in their fight to gain access to the life-saving treatment Soliris.

I DO NOT support PHARMAC's proposal to decline funding for the Soliris treatment.
I support the PNH Support Association's proposition for Equity and Fairness which states:

PHARMAC must return to the negotiating table with the supplier of the Soliris treatment

PHARMAC must negotiate in good faith toward funding Soliris for a minimum of 8 NZ PNH patients

PHARMAC must establish fair assessment criteria, based on expert advice from the international haematological community, to assess patient need for the Soliris treatment

PHARMAC must amend its Operating Policies and Procedures to acknowledge the right of rare disease patients to access life restoring and life-saving treatments as in the specific example of the Soliris treatment

I would be happy to provide further information should this be required.

Yours faithfully

[redacted]

From: Michael Newell [redacted]
Sent: Monday, 29 July 2013 9:39 p.m.
To: OPP Review
Subject: Submission in response to PHARMAC's consultation on Decision Criteria.

To Whom it May Concern;

I support PNH patients in New Zealand in their fight to gain access to the life-saving treatment Soliris.

I DO NOT support PHARMAC's proposal to decline funding for the Soliris treatment.

I support the PNH Support Association's proposition for Equity and Fairness which states:
i. **PHARMAC must return to the negotiating table** with the supplier of the Soliris treatment
ii. **PHARMAC must negotiate in good faith** toward funding Soliris for a minimum of 8 NZ PNH patients
iii. **PHARMAC must establish fair assessment criteria**, based on expert advice from the international haematological community, to assess patient need for the Soliris treatment
iv. **PHARMAC must amend its Operating Policies and Procedures** to acknowledge the right of rare disease patients to access life restoring and life-saving treatments as in the specific example of the Soliris treatment

Yours faithfully,

Mike Newell

From: Renee Manella [redacted]
Sent: Wednesday, 17 July 2013 4:06 p.m.
To: OPP Review
Subject: Submission in response to PHARMAC's consultation on Decision Critera.

To Whom it May Concern;

I support PNH patients in New Zealand in their fight to gain access to the life saving treatment Soliris.

I DO NOT support PHARMAC's proposal to decline funding for the Soliris treatment.

I support the PNH Support Association's proposition for Equity and Fairness which states:

i. **PHARMAC must return to the negotiating table** with the supplier of the Soliris treatment

ii. **PHARMAC must negotiate in good faith** toward funding Soliris for a minimum of 8 NZ PNH patients

iii. **PHARMAC must establish fair assessment criteria**, based on expert advice from the international haematological community, to assess patient need for the Soliris treatment

iv. **PHARMAC must amend its Operating Policies and Procedures** to acknowledge the right of rare disease patients to access life restoring and life saving treatments as in the specific example of the Soliris treatment

I personally find it disgraceful that we are not among the nations who make Soliris available to people suffering from PNH, and find it abhorrent that the excuse proposed is a financial one. It makes a bleak statement about our country and its future if we lack the human compassion to fund this essential treatment.

Yours faithfully

Renee Manella

From: John Forman [redacted]
Sent: Tuesday, 6 August 2013 11:42 a.m.
To: Sue Anne Yee
Subject: FW: Submission on your proposal to decline a funding application for eculizumab

Hi Sue Ann,

A slight amendment in this copy, changes one sentence and adds a reference to the economic evaluation in Appendix 2, as follows.

I have changed the text and highlighted in yellow on the last page.

This is a significant underestimation of survival gain in comparison with the seven year data set from the UK experience, which shows normalised mortality for patients receiving eculizumab. (Kelly R, et al. Long Term Treatment with Eculizumab In Paroxysmal Nocturnal Hemoglobinuria: Sustained Efficacy and Improved Survival. Blood 117[25], 6786-6792. 2011).

Regards, john

John Forman
Executive Director, NZORD
New Zealand Organisation for Rare Disorders
[redacted]
Website: www.nzord.org.nz

From: John Forman
Sent: Tuesday, 6 August 2013 10:00 a.m.
To: 'eculizumabfeedback@pharmac.govt.nz'
Cc: [redacted]
Subject: Submission on your proposal to decline a funding application for eculizumab

Introduction:

This submission is from the NZ Organisation for Rare Disorders (NZORD). We are a charitable trust set up in 2000 to improve information, support, clinical care, treatment and research for rare disorders. We work closely with wider networks of 160 rare disease support groups, and 60 larger groups advocating for those with common disorders.

Thank you for the extension of time (conveyed to us in an email from Dr Sue Anne Yee on 5 June 2013) given to NZORD to prepare this submission on your proposal to decline a funding application for eculizumab.

As mentioned when requesting the time extension, NZORD held a one-day seminar on Thursday 1 August with a range of speakers covering academic and professional experience, patient advocacy, basic science research, media and industry representatives, plus a political panel. Attendance, though varying at different times of the day, was an average of about 65 to 70 people present for all sessions.

This submission reflects presentations and discussions at the seminar, as well as many themes which have emerged from discussions and networking NZORD has done with a variety of patient advocacy groups and other stakeholders (including professional groups, industry, regulators, and payers, both nationally and internationally) over many years relating to development, registration and funding of medicines, and specialised medicines in particular. It is also informed by discussions at several of Pharmac's recent forums on its decision criteria consultation, and advice received from other professional advisors.

We ask Pharmac to consider this submission in the context of a previous request we sent to Pharmac on 29 July 2013, asking Pharmac to withdraw this consultation because of errors and misleading information contained in it. That request should also be considered a submission from NZORD to you on this consultation, and is attached as Appendix 1.

Given your concurrent consultation on your decision criteria, you should also consider this submission to be relevant to and forming a submission on that consultation as well, even though we are likely to submit further on that particular consultation closer to the close of those submissions.

Summary of submission:

- The consultation document contains several significant errors and has other misleading information in it. Pharmac should withdraw the document, alert all stakeholders, and publish an explanation of this.
- In deciding on this matter and any other decisions, Pharmac must act consistently with the human rights framework that exists in New Zealand, including acting equitably and incorporating community values to give practical effect to the right to life and the right to health. There is no indication in the document that any of these issues have been addressed.
- Pharmac must also comply with the principles of NZ's legal and constitutional system, and in this context this means when acting as an agent of the DHBs, placing greater emphasis of the purposes of the Act, the objectives of DHBs, Minister's expectations (specifically about access to specialised medicines), its MoU with DHBs, and policy and decision criteria guidance in the health sector. The document does not address any of these important factors nor discuss how the proposal addresses these objectives and requirements.
- The role of ethics in decisions is an important one but poorly addressed by Pharmac over many years, and not addressed at all in this consultation. Appropriate and proper decision-making in health requires that these issues are more robustly addressed in a way that is consistent with the widely accepted role of ethics in healthcare in NZ.
- Consumer engagement has not been adequately undertaken by Pharmac in respect of this particular decision, and in general Pharmac's consumer engagement falls far short of a standard expected across all areas of life in NZ.
- There are several weaknesses in the economic evaluation done by Pharmac on eculizumab, and failures by PTAC and its subcommittee to adequately address several important decision criteria set out in your operational policies and procedures. Pharmac itself also fails to address several of these criteria in its document.
- There are contested views of Pharmac's interpretation of several key phrases in its legislative brief, including the meaning of "best health outcomes" and "reasonably achievable". We propose alternative perspectives which Pharmac should take into account and which we believe would lead to a different decision on this consultation.

Discussion.

1 – The Human Rights dimension, including the right to life and the right to health.

We submit Pharmac must take into account as an "other criteria" for this consultation, and for all decisions, that within the maximum of available resources (see Article 2 of the International Covenant of Economic, Social and Cultural Rights (ICESCR)), any rationing and priority setting decisions of Pharmac should be consistent with human rights recognised in New Zealand.

This means:

- Pharmac should prefer an interpretation of the New Zealand Public Health and Disability Act 2000 (NZPHD), which includes Pharmac preferring an interpretation of its objectives and functions in section 47 and 48 of the NZPHD Act, that is consistent with the rights and freedoms contained in the New Zealand Bill of Rights Act 1990 (NZBoRA) over any other any other interpretation of the

NZPHD (see section 6 of the NZBoRA).

- Even if the legislative framework is considered by Pharmac to reflect these rights, Pharmac must also take these rights into account when making decisions.

- This means Pharmac must take into account the following rights when making this decision:

    - The inherent dignity of the human person.

    - "[T]he right of everyone to the highest attainable standard of physical and mental health" (see ICESCR, including Article 12.1)

    - The rights and freedoms in the NZBoRA, including:

        - the right not to be deprived of life, except on such grounds as are established by law and are consistent with the principles of fundamental justice (see section 8 of the Bill of Rights Act and Article 6 of the International Covenant on Civil and Political Rights (ICCPR)). This right favours funding life-saving medicines.

        - the right not to be subjected to torture or to cruel, degrading, or disproportionately severe treatment of punishment. In particular, Pharmac should note that for patients declined treatment when a treatment is available in most other comparable countries these patients consider this is cruel treatment by the state.
        - the right to freedom from discrimination, including on the grounds of disability (including physical illness). In particular, patients suffering from PNH disease are indirectly discriminated against on the grounds of rarity of disability. It is known that therapies specifically targeted for rare diseases will have higher cost and less statistically robust efficacy data, and that unless this is recognised by Pharmac in its rationing decisions there will be structural discrimination against those patients, like PNH patients, who suffer from a rare disease.

    - The human right of everyone to the enjoyment of the highest attainable standard of physical and mental health (see UN Special Rapporteur on the Right to Health).

    - The right of each person to share in the benefits of scientific progress (see Article 15 of the Universal Declaration on Human Rights).

    - The right of a person to a standard of living adequate for the health and well-being, medical care and necessary social services, and the right to security in the event of sickness, disability or other lack of livelihood in circumstances beyond a patient's control (see Article 25.1, Universal Declaration on Human Rights).

    - The duty of the state of New Zealand and Pharmac as a public body to protect its citizens. For example:

        - in the third Article of the Treaty of Waitangi the Queen extended her "royal protection and imparts to them all the Rights and Privileges of British Subjects".

        - the duty of the state not to abandon its citizens (often referred to in the health context as the "rule of rescue"). It is noted that the Ombudsman in his recent opinion stated this principle has obvious relevance where there is no known alternative method of treatment (see Ombudsman's opinion).

- To realise these rights, steps must be taken by Pharmac to, within the maximum of available resources, assure all medical service and medical attention in the event of sickness (see Article 12.2 of the ICESCR).

- Pharmac must act equitably. Equity requires that like cases be treated in a like manner, but also that exceptions be treated in an unlike manner. Rigid application of policy results in inequitable outcomes. As Pharmac knows that an overriding cost utility approach puts highly-specialised treatments at a significant disadvantage, it is inequitable for those patients not to consider the broader considerations which are relevant in those exceptional cases.

- Community values (such as the rule of rescue) support that New Zealand is prepared to pay more for specialised medicines for small patient populations than for medicines for the general population.

2 – Pharmac should comply with the basic principles of New Zealand's legal and constitutional system.

This requires Pharmac to exercise its public authority legally, reasonably, and honestly. (See, for example, Chapter 3 of the Legislation Advisory Committee Guidelines).

The interpretation of Pharmac's statutory objectives and functions (see ss 47 and 48 of the NZPHD Act) must take into account the rights expressed above and also the purpose of the NZPHD Act. That Act's purpose includes, to the extent that is reasonably achievable within the funding provided, to achieve for all New Zealanders the improvement, promotion and protection of their health, the promotion of the inclusion and participation in society and independence of people with disabilities, and the best care or support for those in need of services (see section 3 of the NZPHD Act 2000). The purposes of the Act would be met by providing funding.

As Pharmac makes purchasing decisions on behalf of DHBs, Pharmac must also take into account in relation to this decision, which is effectively made as a purchasing agent of a DHB:

- The statutory objectives of DHBs, which include (see section 22 of the NZPHD Act):
    - To improve, promote, and protect the health of people;
    - To reduce health disparities by improving health outcomes for Maori and other population groups (such as PNH patients)
    - To exhibit a sense of social responsibility by having regard to the interests of the people to who it provides, or for whom it arranges the provision of, services
    - To uphold the ethical and quality standards commonly expected of providers or services and of public sector organisations.

- the MOU between Pharmac and DHBs. Notably in clause 3 of the MOU Pharmac agreed that "Equity of access, reducing inequalities and improving health outcomes for individuals and communities will guide our relationship and decision making". "Reducing inequalities" requires more than making decisions which achieve the "greatest good for the greatest number". Reducing health disparities, exhibiting a sense of social responsibility, and upholding the ethical standards expected of a public sector organisation means like cases should be treated alike but different cases should be treated differently.

- The NZ Health Strategy and the NZ Medicine Strategy, among many high level policy guidance documents in health that are derived from the Act, which provide detailed commentary on aims, objectives, outcomes and action points to guide decisions by DHBs, and Pharmac specifically in the case of the Medicine Strategy.

- Government's 2010/11 Letter of Expectations to Pharmac which expected that Pharmac would respond to specific expectations and areas of focus including "Achieving better access to medicines, which will include continuing to work with [the Minister] and key stakeholders around improving access to high cost, highly specialised medicines".

- Decision criteria issued as guidance for the health sector by the National Health Committee (NHC) in 2003, and recently updated by the new NHC, and other decision criteria published by the Ministry of Health (e.g. for the high-cost treatment pool), all of which consistently place "soft" criteria of fairness, equity and community values alongside the "hard" criteria of efficacy, cost-effectiveness and budget management, to achieve a balanced set of decision criteria.

NZORD submits that the eculizumab consultation document fails to adequately comply with these legal and constitutional matters, especially by its failure to address in the document, the variety of other matters that should be considered.

3 – The role of ethics in decision making by Pharmac.

We submit that in order for Pharmac to fulfil its obligations under the human rights approach and to meet its legislative mandate within the principles of New Zealand's legal and constitutional system, it must give specific consideration to moral factors of fairness, equity and community values in this and in all decisions.

In a practical sense, as applied ethics, this becomes the way in which the rest of our health system addresses the right to health and the right to life. We submit that Pharmac has the same duties in this regard as the rest of our health system, and does not have a discretion whether or not to factor such matters into its decisions. In other words, these matters are mandatory considerations for Pharmac under "other criteria" and not discretionary considerations.

In support of this part of our submission we refer to these additional points about the role of fairness, equity and community values as moral factors that help give effect to the human rights dimension, and the legislative and constitutional principles:

A – How Pharmac has itself has addressed these matters:

- Detailed discussion in Pharmac's 2003 policy document, A Prescription for Pharmacoeconomic Analysis (PFPA) v1.1 2003 pp 9 and 10, and elsewhere in the same document, which address the limitations of cost-utility analysis and the need to consider other factors, including equity.

- The 2007 version of PFPA which removed almost all such discussion other than a brief statement that such matters as equity, acceptability and need can be taken into account during the prioritisation and decision-making process.

- The 2012 version of PFPA which retains just a brief statement that: "Other inputs to health decisions, such as equity and social justice, can be considered under Pharmac's other decision criteria."

- Commentary in response to submissions on the review of the exceptional circumstances scheme which resulted in the Named Patient Pharmaceutical Assessment scheme (NPPA) 2011, and in response to the Ombudsman's enquiry into a complaint from ms Freda Evans, where Pharmac makes it clear it sees no place for specific reference to moral factors such as fairness, equity and community values in its decision criteria.

- Direct discussions on several occasions in recent years between Pharmac and NZORD where Pharmac officials have stated, sometimes forcefully, that moral factors have no place in their decision processes.

B – how others see it:

- The Ombudsman in his opinion of May 2013, stating there is a contestable legal argument about Pharmac's obligation to consider such matters. He added that even if Pharmac's suggestion is correct that the values are reflected in the legislative framework, needing Pharmac only to act ethically within that, this does not mean Pharmac is precluded for considering them. The Ombudsman also (as mentioned above) stated that the principle of the state not to abandon its citizens has obvious relevance where there is no known alternative method of treatment.

- Commentary at the NZORD seminar on 1 August from Andrew Moore, one of NZ's most experienced philosophers in the area of health rationing, that Pharmac's insistence that its objective to achieve the "best health outcomes that are reasonably achievable....." translates into an imperative to maximise health gains across the whole population, is in itself based on an ethical view that is contested and controversial. In other words, Pharmac is using its preferred ethical argument as an interpretation of its statutory objective, and to argue against any further consideration of ethical arguments.

NZORD considers that Pharmac has failed in its duty to maintain consistency with the rest of the health sector in its approach to these moral considerations, and in doing so has embarked on a deliberate process of marginalising such considerations from its own decision making, without any transparency about what it has been doing, nor open disclosure to stakeholders or the community (nor, we suspect, to Parliament or Ministers) about its aims or the direction of this policy shift.

In respect of the human rights dimension, legal and constitutional matters, and the place of ethics in decision making in health, we submit that Pharmac has a responsibility to remedy this situation and not proceed with reaching a decision on this particular consultation without first reporting on these matters to the Minister, as matters of high policy needing to be addressed by government and/or Parliament.

4 – Consumer engagement.

NZORD holds the view that as the most affected and most important stakeholders in medicine decisions, (as their health and quality of life is critically at stake in such decisions), patients/consumers have been poorly involved in any aspect of this particular funding decision to date. We consider this poor level of engagement is reflected in other very poor levels of patient/consumer engagement by Pharmac across all of its work generally.

Most of what you do deals with health professionals and industry. A Consumer Advisory committee that advises you primarily on how to engage better with consumers, does not ensure that those patents/consumers with a direct interest in particular health conditions have any planned or systematic opportunities to ensure their interests and concerns are well understood and considered by you when crucial decisions are made.

By contrast, a wide range of public bodies including health providers take on significant responsibility to continually engage with groups and communities that are affected by their decisions, and in line with their consultation obligations, ensure there are specific opportunities for formal engagement when important decisions are looming. This includes allowing them to have direct input into decisions through formal consideration of issues they submit.

Similarly, in other jurisdictions such as Australia and the UK, the equivalent agencies to Pharmac ensure there is specific engagement of patients/consumers prior to or in parallel to the technical evaluation by PTAC or its equivalent. We submit that Pharmac's consultation processes are seriously deficient not only in the case of this particular drug, but across all of your work in medicines generally. We will discuss this more in our submission to your consultation on decision criteria.

5 – Your economic analysis of eculizumab.

Appendix 2 to this report (below) critiques the analysis performed on eculizumab by Pharmac and questions many aspects of the analysis, decision criteria considered, and reporting of the analysis. We believe this criticism should be considered alongside other aspects of this submission which question the validity and accuracy of key parts of the information provided. In our view the information in the appendix strengthens our call for the submission to be withdrawn because of errors and misleading information in it.

6 – Others matters of relevance.

Here are some of the themes discussed among rare disease patient groups and other stakeholders, which challenge the approach usually taken by Pharmac. We see these are providing for a broader and more balanced approach to medicine decisions, and in a way that is more consistent with the decision criteria of DHBs and other parts of our health system, and with the right to health that applies to all New Zealanders.

- Pharmac has a tendency to emphasise the "tough decision" approach that some will be funded and others not. That is too simplistic and can also be unfair. There are very good reasons to adopt an equitable approach that spreads medicine funding across as many areas of health need as possible. For example if there are 15 new medicines waiting for funding but only enough money to fund 10, why not fund all 15 at a level that provides for about 70% of the need for each of the 15 diseases (with some restrictions as to eligibility within each disease category to manage this), rather than having a winners and losers approach that leaves 5 of the diseases out altogether.

- Many groups of patients suffer from very real disadvantage because their condition is very rare, and when treatments become available for them they are doubly affected by 1) the higher cost of new treatments, and 2) the higher cost of a very small "market" for that medicine. There should be some extra consideration given for diseases in this situation so that rare disease patients do not suffer compounded disadvantage. One size does not fit all.

- Ideally there should be an additional layer of decision-making for very rare diseases that do not fit standard cost-effectiveness thresholds for large populations. That additional layer exists in Australia, Scotland and other countries, because they have decided that is a fair way to deal with the disadvantage rare disease faces. NZ should also have weighting built into decision criteria to counter the disadvantage of rarity.

- Pharmac regularly talk about their decision criteria being applied "consistently" so that all applications are evaluated against the same criteria. Pharmac's assumption of fairness in this approach is not correct. This will not protect the right to health of vulnerable small populations, nor produce fair or equitable outcomes for people in a wide variety of circumstances that can be very different according to the wide variety of health conditions, and how frequently they occur in the population. For example, in a recent opinion following a complaint to the Ombudsman about a Pharmac decision, the Ombudsman noted that the decision criteria under the NPPA (exceptional circumstances) scheme ought to be clearly differentiated from those under the pharmaceutical schedule. This opinion demonstrates the inappropriateness of considering medicines for individuals or tiny populations under the same criteria used for large populations.

- Pharmac's assumption that "best health outcomes" as mentioned in its legislative brief, can be strongly associated with calculation of Quality Adjusted Life Years, and opportunity costs, is too narrow a view of what is "best", especially when Pharmac's calculations do not take into account non-health-sector costs. The decision criteria should include a broader range of considerations that are important to patients, so that the decisions are made in a patient-centred way.

- Pharmac is also charged with achieving outcomes that are "reasonably achievable". We believe their narrow perspective on technical assessment and budget management, to the exclusion of patient rights and interests from their decision processes, and outcomes that effectively discriminate against patients with rare diseases, is not a reasonable outcome by any measure.

Applying the principles set out in these points would lead to fairer allocation of funding for all rare diseases and ensure that patients with conditions like PNH would not be arbitrarily abandoned by our health system, in breach of their right to health.

John Forman
Executive Director, NZORD
New Zealand Organisation for Rare Disorders
[redacted]
Website: www.nzord.org.nz

Appendix 1

From: John Forman [redacted]
Sent: Monday, 29 July 2013 9:55 a.m.
To: Steffan Crausaz
Cc: Stuart McLauchlan; Sue Anne Yee; eculizumabfeedback
Subject: Your urgent attention required - Your consultation on a proposal to decline a funding application for

eculizumab
Importance: High

The Chief Executive
Pharmac

Copies to Chair of the Pharmac board and the Eculizumab consultation team

Dear Steffan,

Your urgent attention required - Your consultation on a proposal to decline a funding application for eculizumab

You will be aware of issues NZORD has commented on publicly in a recent press release about errors and misleading information in the consultation document you sent out on 21 May 2013. NZORD was planning on commenting on these matters in our submission on the consultation document. However, the errors are so fundamental that NZORD has decided to formally draw these matters to your attention by way of this separate email.

Pharmac needs to ensure that people you consult with are adequately informed and NZORD considers revised information needs to be made available to submitters to ensure this is the case. We suggest you achieve this by withdrawing the consultation document, reissuing it and extending the consultation period. You could post this letter on your website to explain the extended consultation period. We think you should also draw the revised information to the attention of all of those to whom you sent the 21 May 2013 consultation document.

As you will know, one of the leading court cases on consultation in New Zealand is *Wellington International Airport v Air New Zealand*. That case set out useful statements on the standards for adequate consultation in New Zealand. One of the statements was:

> *Implicit in the concept [of consultation] is a requirement that the party consulted will be (or will be made) adequately informed so as to be able to make intelligent and useful responses. It is also implicit that the party obliged to consult, while quite entitled to have a working plan already in mind, must keep its mind open and be ready to change and even start afresh*

NZORD considers Pharmac needs to correct two fundamental errors in its consultation paper and provide additional information on the funding of eculizumab in other jurisdictions.

The first error is stating a likely treatment group of up to 20 patients. This conflicts with advice from your PTAC committee and their estimate of 3 patients per million, suggesting up to 13 patients in total. Your document exaggerates this by about 55%. The estimate of 13 patients is validated by a pro-rata population-based assessment of number actually treated in Australia, again concluding 13 patients in New Zealand.

The second error is stating the price of eculizumab at "more than $600,000 per patient per year" when it is known both to Pharmac and to NZORD that a confidential offer has been made for a discounted price to treat New Zealand patients. Though NZORD does not know the detail of this offer, it is clear that the cost is exaggerated in your document.

The combination of these two figures produces a cost of "approximately $12,000,000 (20 patients) per year" if eculizumab was funded. This cost estimate is a misleading statement. The calculation provides a figure for the treatment of patients that we understand overstates the costs (if you decided to fund treatment) by more than 100%.

Further we consider the statements from PTAC minutes regarding funding in Canada and Scotland are referred to in a misleading manner. Pharmac has relied upon the minutes for advice regarding eculizumab and has drawn the minutes to the attention of submitters via a prominent link on the website page about the consultation. See http://www.pharmac.health.nz/news/item/proposal-to-decline-a-funding-application-for-eculizumab (accessed online 22 May 2012 and again 29 July 2013).

The PTAC minutes of August 2012 at para 3.11 discuss the drug's high cost and poor cost-effectiveness: "The Subcommittee noted that this is the reason why the Canadian Agency for Drugs and Technologies in Health (CADTH) and Scottish Medicines Consortium did not recommend it for use within their jurisdictions." Pharmac refers to the PTAC recommendations about cost and cost-effectiveness as part of its own reasoning in the consultation document. However neither PTAC, in those or their subsequent March 2013 minutes, nor Pharmac in their consultation document, make note of the fact that subsequent decisions in Canada have seen the drug funded in most parts of that country. There is also no reference to the Scottish government taking steps to set up a special fund for orphan drugs for rare diseases, in part because of the equity issues raised by the recommendations not to fund eculizumab, and have commenced funding patients there.

In failing to present the other side of the story about the position in Canada and Scotland, Pharmac will have misled many submitters as to funding decisions made in other jurisdictions.

NZORD considers these errors and misleading statements result in the consultation paper not suitably informing submitters. The errors seriously prejudice the interests of the patients who are seeking treatment with eculizumab and will have mislead potential submitters who might have relied wholly on the information Pharmac has provided when forming their views and making a decision on whether to provide a submission.

We believe Pharmac has a duty to correct the information and start the consultation afresh.

Please advise us as soon as possible what you intend to do to in response to this request. We are happy to discuss this with you further if that would assist.

Please note this email only focuses on those matters we consider require urgent attention to ensure Pharmac's consultation process is valid. This email does not describe all of NZORD's views on the consultation document. We consider other factors are also relevant to your decision (such as the right each patient has to health, and other community values). We intend to make a fuller submission (on a revised consultation document) in due course.

Yours sincerely,
john

John Forman
Executive Director, NZORD

Appendix 2

Analysis of Pharmac consultation document for eculizumab, including comment on recorded information from PTAC and the Haematology subcommittee, and addressing decision criteria, economic evaluation, and the reporting of the analysis. Prepared by Alison Davies, Health economist.

Decision Criteria Used to Make this Decision

I note that advice was received from PTAC and the Haematology SC – this was used to provide advice and Pharmac also summarise the reasons why they propose to decline funding.

According to the Minutes, PTAC considered the following factors relevant in giving a recommendation to decline: 5 criteria i.e. 1, 3, 4, 5 and 6 – they did not consider the following criteria 2, 7, 8 and 9 relevant. Therefore they did not consider Maori/Pacific (2), cost to patient (7) or government health priorities (8) –or other (9). Cost to Health Service Users would appear to be particularly important (given the cost of this treatment) but was not considered relevant to the decision – this appears to be a flaw in decision making process as perhaps the most important criterion was not included i.e. that patients cannot afford this medication. Arguably under Criterion 9, other issues (see below) should have been included.

According to the Minutes of the Haematology SC, they considered only 4 criteria relevant to their recommendation (1,3,4,6). This means they did not consider 2,5,7,8,or 9 relevant. Looking at these individual criteria, they did not consider Maori/Pacific (2), Cost-Effectiveness (5), cost to patient (7), Government Priorities for Health Funding (8) or Other (9). I would think that Decision Criteria 5, 7, 8 and 9 are relevant to the decision and it is a defect in the decision making process that they were apparently not taken into account or considered relevant.

Pharmac's expert advice therefore does not include advice on what appear to be particularly relevant criteria to the decision. Criterion 7 would appear to be particularly relevant to this decision as the cost of this medication to patients is prohibitive and beyond the scope of almost all NZers because of its high cost, this should have been considered. Given that the proposal to decline funding effectively deprives the patients concerned of their right to life, then the consequences of not funding and specifically the ethical and legal implications (e.g. under Human Rights obligations) of this need to be taken into account as other factors in the decision. These are not within the decision criteria that Pharmac ordinarily considers and therefore should have been consulted on.

Economic Evaluation

I note that the economic evaluation (TAR 209) is dated May 2013 – therefore this was not available to PTAC (or the Haematology SC) when they gave Pharmac advice (Feb 2012, Aug 2012, Feb 2013 and March 2013). Therefore, this begs the question how Cost Effectiveness was taken into account by PTAC? There does not appear to be any evidence that the cost utility analysis prepared by Pharmac (TAR 209) was considered by the expert advisers (either PTAC or the ESC). There is no explicit discussion in the minutes about the analysis and no peer review of it by the relevant committees.

The report

The CUA is presented in TAR 209. Comments on this are as follows:

- The scope of the analysis is "preliminary" therefore the analysis was quite cursory in nature – undertaken in 1-2 weeks. According to PFPA, "Preliminary Assessment largely using opportunistic data. Rapid systematic review of evidence undertaken. May require further modelling compared with a rapid CUA (due to disease complexity, risk, or uncertainty of results). Reviewed internally. FTE required 2-4 weeks"

- The report does not provide sufficient detail to assess how the analysis was actually carried out – i.e. there is not a clear description of methods and inputs or even outputs of the analysis. Based on the superficial nature of the analysis, the 2-4 weeks would have been spent writing up the report rather than undertaking the analysis itself. This represents a "back of the envelope" calculation which becomes evident when the analysis is examined in more detail (or at least as much detail as can be gleaned from the report). PFPA states It is important that CUAs are transparent so that quality and validity can be assessed.

- The report describes best supportive care (BSC) but does not appear to calculate the costs associated with best supportive care. For example, there is no detail on resources used and unit costs associated with BSC. The costs associated with best supportive care appear to be dismissed as small (in comparison with drug cost) and therefore not relevant. The report states that Alexion did not supply a cost utility analysis (Page 4) yet the report also states that the company's cost offsets (Page 22) were small. There is no disclosure of the inputs or results of the Alexion analysis and how they differed from the Pharmac analysis.

- The summary does not report the resource use or costs in any detail (either aggregated or disaggregated) so it appears that the cost of eculizumab is the only cost used in the analysis and best supportive care has no cost associated with it (?).

- As for the cost of the eculizumab, the cost has been calculated at $685,000 in the 1st year and $670,000 per year thereafter. The duration of treatment is said to be "whilst they receive benefit from the treatment". However it has been assumed that there is 100% adherence and all patients therefore receive the maximum duration of 25 years. This could clearly serve to overestimate the cost of treatment.

- It is not clear whether discounts (if any) offered by the company have been taken into account in calculating the cost of treatment. PFPA states that "For pharmaceuticals listed on the Pharmaceutical Schedule, the price of the pharmaceutical should include any rebate that has been negotiated with the Supplier. The analysis should state whether the price is confidential."

- There is no adjustment in price for patent expiry. PFPA states "When calculating pharmaceutical costs, consideration should also be given to the length of the pharmaceutical patent and time until a generic pharmaceutical is likely to become available. It is recommended that in cases where the patent expiry is within 10 years from expected date of pharmaceutical funding, the expected time and price reduction from a likely generic pharmaceutical should be included in the analysis. If the patent expiry is after 10 years from expected date of funding, a conservative proxy should be used for the estimated time until the introduction of a generic pharmaceutical and subsequent price reduction (e.g. 25 years until expiry and 70% price reduction with introduction of generic)."

- The calculation of the QALYs generated with treatment versus best supportive care includes an incremental survival gain with treatment of 5 years. (20 year survival with BSC vs. 25 years with eculizumab). This is a significant underestimation of survival gain in comparison with the seven year data set from the UK experience, which shows normalised mortality for patients receiving eculizumab. (Kelly R, et al. Long Term Treatment with Eculizumab In Paroxysmal Nocturnal Hemoglobinuria: Sustained Efficacy and Improved Survival. Blood 117[25], 6786-6792. 2011. In addition, the mean utility over 20 years with BSC is 0.535 –estimated from clinical opinion. Whose clinical opinion has not been specified, also how one utility weight (from one EQ5D score (2,1,2,2,2)) has been averaged over a 20 year period. These values therefore represent a cursory and superficial estimate for disutility which may not be reasonable. There appears to have been no peer review and no attempt to reconcile these values with the literature or weights used in other analyses.

- The results (Section 4.5) present an ICUR (range) but do not show the costs and outcomes for each of the alternatives. PFPA sets out requirements for reporting that have clearly not been met (See Table 12, Page 54).

- The results report that "this is based on incremental gains of 9.2 QALYs with incremental costs of $11.4m over a lifetime. Calculation of the point estimate for the incremental ratio – should result in **$1.2m per QALY or 0.8 QALYs per $1m invested**. The range given in the results therefore misrepresents ICUR ranges by using $1.1m to $2.4m per incremental QALY and 0.4 to 0.9 QALYs per $1m.

- In summary, the analysis undertaken appears superficial and poorly reported. Although it might be argued that adjustments would not make much difference to the overall conclusion, the importance which appears to have been attached to cost-effectiveness as one of the key Decision Criteria in the decision on eculizumab and the consequences of the decision to patients would appear to necessitate a more rigorous approach.

Alison Davies

[redacted]

From: Abraham Batlajeri [redacted]
Sent: Sunday, 2 June 2013 7:01 p.m.
To: eculizumabfeedback

To Whom it May Concern;

I support PNH patients in New Zealand in their fight to gain access to the life saving treatment Soliris.

I DO NOT support PHARMAC's proposal to decline funding for the Soliris treatment.

I support the PNH Support Association's proposition for Equity and Fairness which states:

i. PHARMAC must return to the negotiating table with the supplier of the Soliris treatment

ii. PHARMAC must negotiate in good faith toward funding Soliris for a minimum of 8 NZ PNH patients

iii. PHARMAC must establish fair assessment criteria, based on expert advice from the international haematological community, to assess patient need for the Soliris treatment

iv. PHARMAC must amend its Operating Policies and Procedures to acknowledge the right of rare disease patients to access life restoring and life saving treatments as in the specific example of the Soliris treatment

It is simply unacceptable for patients to die because a life saving treatment is being refused to them!!

Yours faithfully,

Abraham Batlajeri

From: Ian & Alice Williamson [redacted]
Sent: Thursday, 20 June 2013 7:40 p.m.
To: eculizumabfeedback
Subject: Proposal to decline funding for Soliris

Dear Pharmac

Please reconsider your proposal to decline this drug, when you know of cases in NZ where it could provide people with life / hope. The Medical professions job is to save lifes. As you are part of this profession you are acting in direct contradiction to this ethical requirement.

Fund the medication. Do the right thing.

Alice Williamson, Longburn, Manawatu
[redacted]

From: Alison elizabeth Danks [redacted]
Sent: Monday, 29 July 2013 8:10 a.m.
To: eculizumabfeedback
Subject: Soliris

Please allow the use of this important drug to help sufferers lead a better quality of life.

Thanks
Alison

From: [redacted]
Sent: Tuesday, 30 July 2013 4:07 p.m.
To: eculizumabfeedback
Subject: For [redacted].docx
Attachments: For [redacted].docx; ATT00001.txt

Submission Regarding the Decision by PHARMAC

to Decline Funding for the Drug Eculizumab (Soliris)

[redacted] is our amazing cousin. We say amazing because not only is she an awesome person, but we absolutely admire the way she lives her life despite living with PNH.

[redacted] She just gets on as best she can - because she has no choice. Unless of course, PHARMAC overturn their negative decision to fund the life changing drug eculizumab.

Our whole family is understandably very concerned about [redacted]'s situation. How she copes, we do not know. [redacted]

This submission is the only way we can try to help her. So please let it be that it does not "fall on deaf ears". Make it an effective submission that helps eculizumab become part of normal dispensing for PNH sufferers.

We find it totally incredulous that the only way [redacted] can access the drug is for her to move to Australia – WHAT?! For PHARMAC to effectively send sufferers away from their own country and even worse, away from friends and family when they need them most, just doesn't make any moral sense at all (and in reality, will Australia be delighted to pick up the bill? You can be pretty sure they will eventually close the gate!).

Thank you for allowing us the opportunity to point out that this:

CANNOT BE A DECISION BASED ON PHARMAC's BOTTOM LINE, IT CAN ONLY BE A MORAL DECISION.

Eculizumab is a life changing drug and it is available for purchase.

[redacted]

PS

Just a suggestion – apply, or plead, beg, whatever it takes, to central government for increased funding so that PNH patients can live without fear and families can be saved sadness and heart break that is unquestionably avoidable.

Sent from my iPhone

From: Steve and Allyson Lock [redacted]
Sent: Tuesday, 2 July 2013 10:25 a.m.
To: eculizumabfeedback
Cc: John Key (MIN)
Subject: Funding of eculizumab

Dear Sue Anne Yee

I am writing to you regarding the proposal to decline funding for eculizumab.

Firstly, i note that you ask to **"hear views as to whether any decision criteria (other than the eight we regularly consider) should be taken into account when deciding this application and, if so, what they should be."** This is interesting to me, because I know that PHARMAC have nine decision criteria that they consider. Which one have you conveniently not told the uninitiated about?

Secondly, **"PHARMAC's cost-utility analysis of the use of eculizumab in patients who have PNH shows that eculizumab is not very cost-effective compared with other funding options".** Would you please describe to me exactly what other funding options there are for PNH?

"PHARMAC's current view is that we cannot justify progressing eculizumab for funding in light of other funding options for DHBs. By way of example, in the 2011/12 financial year, we estimate that $5.5 million was spent on new community medicine investments which benefitted approximately 19,000 patients. A decision to fund eculizumab for 12-20 patients from 2013/14 onwards would potentially mean that 40,000 other patients would be missing out on health gains (from other treatments that could be funded instead)." Interesting. Was the extra money invested (although $5.5 million is a scant amount, approximately $1.25 per person per head of population) in already available medicines? Or was it on new medicines for people who have had no access to treatments for fatal diseases? You could be talking about supplying a few extra boxes of panadol per hospital around the country. After all, providing headache tablets vs. a medication which will save someone's life... well, we really need to look at that don't we? Please make it clear what extras you are funding before you ask people to comment on it.

"We recognise that a decline proposal is not what patients with PNH are hoping for, but we are proposing to decline this funding application because we understand that certainty is something that patients with PNH and their families would prefer." Please don't make it sound as though you are doing PNH sufferers and their families a favour by saying this. What they would "prefer" is funding of treatment for their terrible disease. How much more condescending and patronising could you possibly be?

"If the PHARMAC Board makes a decision to decline this funding application, it would mean that PHARMAC would not progress eculizumab for funding. However, if such a decision was made, it would not prevent PHARMAC from reconsidering funding for this treatment in the future if (for instance) material new evidence became available or if the price reduced substantially." Let's investigate this paragraph briefly. PHARMAC have admitted this medication does work, it is the cost that is the reason for not funding. So why don't you just say "If the price comes down, we may consider it". That would be the honest thing to say. We know that PHARMAC puts a price on peoples heads, just be up front about this. Just say "you are not worth spending that sort of money on". Simple.

We want to hear from the community on its views about whether it would be appropriate to decline the funding of eculizumab. I say it is NOT appropriate for PHARMAC to decline funding of eculizumab, because to do so, will be to commit the sufferers of PNH to an early death. Euthanasia is illegal in this country, and so is murder i believe? What gives PHARMAC the right to remove our right to life? I also believe this is a Human Rights issue, and I think it is time New Zealanders became more aware of what PHARMAC is doing to New Zealand citizens.

We are also interested to hear views as to whether any decision criteria (other than the eight we regularly consider) should be taken into account when deciding this application and, if so, what they should be.

1. The right to life.
2. The cost of "other funding options" **IF, and i repeat, IF,** they give the same outcome. It is simply not good enough to say that "while we won't fund this medicine to save your life, we will fund your hospitalisation and palliative care until you die". How is that a good comparison?
3. The burden, physically and mentally to families of NOT treating a patient.
4. The financial cost to families of NOT treating a patient. i.e If the patient is a mother of young children, and she dies, will the husband have to give up work to take care of the children, and if so, how do they pay for the mortgage etc? Welfare? If the patient is a man who works, and he dies and leaves a family behind, who looks after his family thereafter? Welfare? Children growing up without a Mum or Dad, parents losing their children. Husbands losing wives, wives losing husbands. THESE are the things you should take into account. Not whether or not people fit into your flawed QALY system!

I look forward to hearing from you regarding my views.

Sincerely

Allyson Lock
President
New Zealand Pompe Network

Proposal to decline a funding application for eculizumab

21 May 2013

PHARMAC is seeking feedback on a proposal to decline the application from Alexion Pharmaceuticals for funding eculizumab (Soliris) for the treatment of paroxysmal nocturnal haemoglobinuria.

Background information relevant to the proposal can be found in the documents linked below.

Why is PHARMAC proposing to decline this funding application?

This proposal to decline the funding application is consistent with the clinical advice we have received, which recommended that the application be declined because although it is an effective treatment, it is extremely expensive.

PHARMAC's cost-utility analysis of the use of eculizumab in patients who have PNH shows that eculizumab is not very cost-effective compared with other funding options. The reason PHARMAC is proposing to decline funding is because the price requested by the supplier is extreme and, given the available budget, appears to be out of reach.

There are always more medicine funding applications than the available budget will allow. Even if DHBs had much more money available, at the current price, eculizumab is not cost-effective and would be likely to be at the back of the queue of medicines that could be funded.
PHARMAC's current view is that we cannot justify progressing eculizumab for funding in light of other funding options for DHBs. By way of example, in the 2011/12 financial year, we estimate that $5.5 million was spent on new community medicine investments which benefitted approximately 19,000 patients. A decision to fund eculizumab for 12-20 patients from 2013/14 onwards would potentially mean that 40,000 other patients would be missing out on health gains (from other treatments that could be funded instead).

We recognise that a decline proposal is not what patients with PNH are hoping for, but we are proposing to decline this funding application because we understand that certainty is something that patients with PNH and their families would prefer.

What does a proposal to 'decline' this funding application mean?

PHARMAC has not made a decision about the funding of eculizumab.

Before we do, we are making information available that we have based this proposal on for people to consider and comment on in detail. We want to hear from the community on its views about whether it would be appropriate to decline the funding of eculizumab. We are also interested to hear views as to whether any decision criteria (other than the eight we regularly consider) should be taken into account when deciding this application and, if so, what they should be.

All consultation responses received for this proposal will be provided to the PHARMAC Board for consideration when it makes a decision on the funding of eculizumab for PNH.

If the PHARMAC Board makes a decision to decline this funding application, it would mean that PHARMAC would not progress eculizumab for funding. However, if such a decision was made, it would not prevent PHARMAC from reconsidering funding for this treatment in the future if (for instance) material new evidence became available or if the price reduced substantially.

Feedback sought

PHARMAC welcomes feedback on this proposal. Your response may include any material which is relevant to the proposal.

Written feedback

To provide feedback, please submit it in writing by **Wednesday, 31 July 2013** to:

Sue Anne Yee
Therapeutic Group Manager
PHARMAC

Email: eculizumabfeedback@pharmac.govt.nz
Fax: 04 460 4995
Post: PO Box 10 254, Wellington 6143

All feedback received before the closing date will be considered by PHARMAC's Board 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.

Consultation meetings

In addition to providing feedback in writing, PHARMAC would welcome requests to discuss this proposal face-to-face with any interested parties. Please forward meeting requests to the email address above.

From: Amy Brooke [redacted]
Sent: Friday, 31 May 2013 3:53 p.m.
To: eculizumabfeedback
Subject: Fund them

Do not decline funding for these people. Barbaric and discriminatory.

Amy Brooke
www.100days.co.nz
Visit my home-page and children's literature website: www.amybrooke.co.nz
www.summersounds.co.nz
http://brookeonline.livejournal.com

100 DAYS
CLAIMING BACK
NEW ZEALAND
www.100days.co.nz

From: Andrew Farrell [redacted]
Sent: Friday, 19 July 2013 12:22 p.m.
To: eculizumabfeedback
Subject: Submission to PHARAMC'S proposal to decline a funding application for Soliris (eculizumab)

Sue Anne Yee
Therapeutic Group Manager
PHARMAC

I DO NOT support Pharmac's proposed intent to decline treatment for PNH

The figures you supply for the number of affected people and cost are in my opinion completely inaccurate and intentionally mislead the public as to possible cost of the treatment. Your intention can only be to skew responses in favour of your proposal. Unethical and disgusting.

Pharmac should consider the patients rights for healthcare, placing so much emphasis on cost and cost effectiveness is putting a price tag on the patients life.

Just because a condition is rare it should not affect the patients access to treatment.

"Equity of access, reducing inequalities and improving health outcomes for individuals and communities will guide our relationship and decision making", This is set out in the agreement between DHBs and Pharmac.

I believe your proposal does not fulfil your duty and agreement and will close the doors for many other patients needing similar treatments down the track.

Also it is not fair to use the same decision criteria as applied to large groups of affected patients. Patients affected by rare diseases are small in number and are unfairly disadvantaged by the high cost of treatment.
The same rules should not be applied when making decisions like this and many overseas countries have already realised this and apply special rules in these circumstances.

I am really disappointed and feel you are failing in your duties.

Regards,
Andrew

From: Andrew Wisker [redacted]
Sent: Sunday, 2 June 2013 8:43 a.m.
To: eculizumabfeedback
Subject: Soliris treatment

To Whom it May Concern;

I support PNH patients in New Zealand in their fight to gain access to the life saving treatment Soliris.

I DO NOT support PHARMAC's proposal to decline funding for the Soliris treatment.

I support the PNH Support Association's proposition for Equity and Fairness which states:

i.
PHARMAC must return to the negotiating table with the supplier of the Soliris treatment

ii.
PHARMAC must negotiate in good faith toward funding Soliris for a minimum of 8 NZ PNH patients

iii.
PHARMAC must establish fair assessment criteria, based on expert advice from the international haematological community, to assess patient need for the Soliris treatment

iv.
PHARMAC must amend its Operating Policies and Procedures to acknowledge the right of rare disease patients to access life restoring and life saving treatments as in the specific example of the Soliris treatment

Please help

A Wisker
Sent from my iPad

From:	Mark & Anna Stewart [redacted]
Sent:	Friday, 31 May 2013 8:35 p.m.
To:	eculizumabfeedback
Subject:	Submission to PHARMAC's Proposal to decline a funding application for Soliris (eculizumab)

To Whom it May Concern;

I support PNH patients in New Zealand in their fight to gain access to the life saving treatment Soliris.

I DO NOT support PHARMAC's proposal to decline funding for the Soliris treatment.

I support the PNH Support Association's proposition for Equity and Fairness which states:

i. **PHARMAC must return to the negotiating table** with the supplier of the Soliris treatment

ii. **PHARMAC must negotiate in good faith** toward funding Soliris for a minimum of 8 NZ PNH patients

iii. **PHARMAC must establish fair assessment criteria**, based on expert advice from the international haematological community, to assess patient need for the Soliris treatment

iv. **PHARMAC must amend its Operating Policies and Procedures** to acknowledge the right of rare disease patients to access life restoring and life saving treatments as in the specific example of the Soliris treatment

Please act for those who cannot help themselves, through no fault or negligence of their own. Its not like smoking, where people deliberately, knowingly and recklessly harm themselves and yet smokers receive subsidies to help them.

NZ needs you to listen. We are counting on you to hear us.

Yours faithfully

Anna Stewart

[redacted]

From: Anne Carroll [redacted]
Sent: Wednesday, 29 May 2013 3:17 a.m.
To: eculizumabfeedback
Subject: pnh - solaris - expression of opinion

To Whom it May Concern;

I support PNH patients in New Zealand in their fight to gain access to the life saving treatment Soliris.

I DO NOT support PHARMAC's proposal to decline funding for the Soliris treatment.

I support the PNH Support Association's proposition for Equity and Fairness which states:
PHARMAC must return to the negotiating table with the supplier of the Soliris treatment

PHARMAC must negotiate in good faith toward funding Soliris for a minimum of 8 NZ PNH patients

PHARMAC must establish fair assessment criteria, based on expert advice from the international haematological community, to assess patient need for the Soliris treatment

PHARMAC must amend its Operating Policies and Procedures to acknowledge the right of rare disease patients to access life restoring and life saving treatments as in the specific example of the Soliris treatment

I currently live in the UK and have a friend here who has pnh. She is being encouraged actively by the NHS here to take solaris for her condition, which she will likely soon do. I am astounded that PHARMAC is taking this stance in such stark contrast. At the very least, we should have some transparency as to the source of information upon which you are purporting to make this decision - and a response to the claims that it is inaccurate.

Yours Faithfully

Anne Carroll

From: Anne Sheehan [redacted]
Sent: Sunday, 28 July 2013 12:58 p.m.
To: eculizumabfeedback
Subject: Pharmac funding of Soliris treatment

To Whom it May Concern;

I support PNH patients in New Zealand in their fight to gain access to the life-saving treatment Soliris.

I DO NOT support PHARMAC's proposal to decline funding for the Soliris treatment.

I support the PNH Support Association's proposition for Equity and Fairness which states:

i. **PHARMAC must return to the negotiating table with the supplier of the Soliris treatment**

ii. **PHARMAC must negotiate in good faith toward funding Soliris for a minimum of 8 NZ PNH patients**

iii. **PHARMAC must establish fair assessment criteria, based on expert advice from the international haematological community, to assess patient need for the Soliris treatment**

iv. **PHARMAC must amend its Operating Policies and Procedures to acknowledge the right of rare disease patients to access life restoring and life-saving treatments as in the specific example of the Soliris treatment**

(Yours faithfully

Anne Sheehan

[redacted]

[redacted]

[redacted]

From: [redacted]
Sent: Tuesday, 4 June 2013 11:37 a.m.
To: eculizumabfeedback
Subject: Submission to PHARMAC's Proposal to decline a funding application for Soliris (eculizumab)

Importance: High

To Whom it May Concern;

I support PNH patients in New Zealand in their fight to gain access to the life saving treatment Soliris.

I DO NOT support PHARMAC's proposal to decline funding for the Soliris treatment.

I support the PNH Support Association's proposition for Equity and Fairness which states:

i. **PHARMAC must return to the negotiating table** with the supplier of the Soliris treatment

ii. **PHARMAC must negotiate in good faith** toward funding Soliris for a minimum of 8 NZ PNH patients

iii. **PHARMAC must establish fair assessment criteria**, based on expert advice from the international haematological community, to assess patient need for the Soliris treatment

iv. **PHARMAC must amend its Operating Policies and Procedures** to acknowledge the right of rare disease patients to access life restoring and life saving treatments as in the specific example of the Soliris treatment

We are parents with a special needs child. We would be further devastated if our daughter needed medical intervention and it wasn't available to her. You have a social responsibility to help improve the quality of people's lives and where possible to improve their life expectancy. Yes, you have a social responsibility. People are not just numbers or statistics. They could be your mother, your friend, your workmate. Would you abandon them? Please help this small group of New Zealanders.

[redacted]

AUCKLAND WOMEN'S HEALTH COUNCIL

SUBMISSION ON

PROPOSAL TO DECLINE A FUNDING APPLICATION FOR ECULIZUMAB

28 July 2013

To: Sue Anne Yee
Therapeutic Group Manager
PHARMAC
PO Box 10-254
WELLINGTON 6143

From: Auckland Women's Health Council
[redacted]

Introduction

The Auckland Women's Health Council (AWHC) is an umbrella organisation for individual women and women's groups in the Auckland region who have a commitment to women's health issues. The focus of the Council is broad and spans many issues that are of interest to women, particularly those that impact on their health and the health of their families. The Council has a special interest in consumer rights, advocacy, ethics committees, medical ethics, the National Cervical Screening Programme, the Code of Consumers' Rights, advocacy services, the Office of the Health & Disability Commissioner and other issues that arose out of the Cartwright Inquiry.

In recent years, the AWHC has also become concerned about the role of the pharmaceutical industry and its effect on a variety of women's health issues.

General Comments

The AWHC has had an interest in the funding of expensive pharmaceuticals since the controversy over the funding of Herceptin became front page news about seven or eight years ago. Around this time the Council became much more aware of issues surrounding clinical trials, the publication of research results, how decisions are made around the funding of new drugs, and the advice provided to the government and health authorities on the benefits, risks and effectiveness of all drugs.

The AWHC is therefore pleased to have the opportunity to comment on PHARMAC's proposal to decline a funding application for eculizumab.

The Council supports PHARMAC's funding process, and the three assessment areas comprising clinical, economic and commercial evaluations that PHARMAC uses to make decisions on the funding of all pharmaceuticals.

Specific issues

1. The cost of the eculizumab.

The AWHC is very concerned at the increasing ability of the pharmaceutical industry to charge exorbitant prices for the some of the niche drugs that various drug companies develop and then vigorously market to the public. Eculizumab is certainly an extreme example of this. We would therefore support a decision to decline the funding application for eculizumab, which has been described as the world's most expensive drug.

2. Current options for the treatment of PNH.

The Council's recommendation that funding for eculizumab be declined is also based on the fact that eculizumab is not a cure for paroxysmal nocturnal haemoglobinuria (PNH), that funding of this drug would be needed long term for those who need treatment, and that there are currently other treatments available and being used in New Zealand for PNH.

3. Drug company funded support groups

The AWHC is alarmed by the growing practice of drug companies' establishing, or helping to set up patient support groups, and then providing funding for such groups to enable them to lobby hard and publicly for a particular drug. This unacceptable use of vulnerable people to publicly pressure governments and health agencies to fund expensive or over-priced new drugs must be exposed and rebutted wherever and whenever it occurs. Alexion Pharmaceutical's funding for PNH patient support groups is widely recognised and the funding it has given to the New Zealand PNH support group is a matter of public record.

4. The use of pharmaceutical funds

We are also concerned at the prospect that funding for eculizumab would mean that hundreds and maybe thousands of other New Zealanders would not have access to the less expensive medications they need. In order to address this issue, the AWHC would support the setting up of a separate source of funding for patients with rare conditions who want access to very expensive drugs.

Conclusion

The AWHC recommends that PHARMAC declines the funding application for eculizumab.

Lynda Williams

On behalf of the Auckland Women's Health Council

From: Barbara Little [redacted]
Sent: Tuesday, 16 July 2013 10:04 p.m.
To: eculizumabfeedback
Subject: Re: Submission opposing PHARMAC's Proposal to decline a funding application for Soliris (eculizumab)

To Whom it May Concern;

I SUPPORT PNH patients in New Zealand in their fight to gain access to the lifesaving treatment Soliris.
I OPPOSE PHARMAC's proposal to decline funding for the Soliris treatment
I support the PNH Support Association's proposition for Equity and Fairness which states:

1. **PHARMAC must return to the negotiating table** with the supplier of the Soliris treatment
2. **PHARMAC must negotiate in good faith** toward funding Soliris for a minimum of 8 NZ PNH patients
3. **PHARMAC must establish fair assessment criteria**, based on expert advice from the international haematological community, to assess patient need for the Soliris treatment
4. **PHARMAC must amend its Operating Policies and Procedures** to acknowledge the right of rare disease patients to access life restoring and lifesaving treatments as in the specific example of the Soliris treatment

If you refuse to fund the life saving treatment you are an effect imposing their premature death. I would be sadden if that was some one I loved dearly with this illness and would do everything to my power to save their life. Please reconsider funding for this life saving medication as it is the right thing we as country should be doing.

Yours faithfully

Barbara Little

July 2013 PNHSANZ*

From: Baukje Lenting [redacted]
Sent: Wednesday, 12 June 2013 6:04 p.m.
To: eculizumabfeedback
Subject: Submission to PHARMAC's Proposal to decline a funding application for Soliris (eculizumab)

To Whom it May Concern;

I support PNH patients in New Zealand in their fight to gain access to the life saving treatment Soliris.

I DO NOT support PHARMAC's proposal to decline funding for the Soliris treatment.

I support the PNH Support Association's proposition for Equity and Fairness which states:

i. **PHARMAC must return to the negotiating table** with the supplier of the Soliris treatment

ii. **PHARMAC must negotiate in good faith** toward funding Soliris for a minimum of 8 NZ PNH patients

iii. **PHARMAC must establish fair assessment criteria**, based on expert advice from the international haematological community, to assess patient need for the Soliris treatment

iv. **PHARMAC must amend its Operating Policies and Procedures** to acknowledge the right of rare disease patients to access life restoring and life saving treatments as in the specific example of the Soliris treatment

Yours faithfully

Baukje Lenting

From: Benjamin Pocock [redacted]
Sent: Tuesday, 16 July 2013 8:44 a.m.
To: eculizumabfeedback; OPP Review
Subject: Re: Submission opposing PHARMAC's Proposal to decline a funding application for Soliris (eculizumab)

To Whom it May Concern;

I SUPPORT PNH patients in New Zealand in their fight to gain access to the lifesaving treatment Soliris.

I OPPOSE PHARMAC's proposal to decline funding for the Soliris treatment.

I support the PNH Support Association's proposition for Equity and Fairness which states:

i. **PHARMAC must return to the negotiating table** with the supplier of the Soliris treatment

ii. **PHARMAC must negotiate in good faith** toward funding Soliris for a minimum of 8 NZ PNH patients

iii. **PHARMAC must establish fair assessment criteria**, based on expert advice from the international haematological community, to assess patient need for the Soliris treatment

iv. **PHARMAC must amend its Operating Policies and Procedures** to acknowledge the right of rare disease patients to access life restoring and lifesaving treatments as in the specific example of the Soliris treatment

I am good friends with the family of one PNH patient and while it seems like a disease that directly affects only a few people, it actually affects many more of us indirectly. I am emailing this submission in support of my friends directly involved. Please do what you can to make sure any avoidable suffering is avoided.

Yours faithfully,

Ben Pocock.

From: Blake and Melanie Old [redacted]
Sent: Sunday, 2 June 2013 2:31 p.m.
To: eculizumabfeedback
Subject: Submission to PHARMAC's Proposal to decline a funding application for Soliris (eculizumab)

To Whom it May Concern;

I support PNH patients in New Zealand in their fight to gain access to the life saving treatment Soliris.

I DO NOT support PHARMAC's proposal to decline funding for the Soliris treatment.

I support the PNH Support Association's proposition for Equity and Fairness which states:

i. **PHARMAC must return to the negotiating table** with the supplier of the Soliris treatment

ii. **PHARMAC must negotiate in good faith** toward funding Soliris for a minimum of 8 NZ PNH patients

iii. **PHARMAC must establish fair assessment criteria**, based on expert advice from the international haematological community, to assess patient need for the Soliris treatment

iv. **PHARMAC must amend its Operating Policies and Procedures** to acknowledge the right of rare disease patients to access life restoring and life saving treatments as in the specific example of the Soliris treatment

Yours faithfully

From: Dominique Cooper [redacted]
Sent: Friday, 31 May 2013 9:52 p.m.
To: eculizumabfeedback
Subject: Submissions in response to PHARMAC's Proposal to Decline Funding for Soliris (eculizumab)

Friday, 31st May 2013

To Whom it May Concern;

I support PNH patients in New Zealand in their fight to gain access to the life saving treatment Soliris.

I DO NOT support PHARMAC's proposal to decline funding for the Soliris treatment.

I support the PNH Support Association's proposition for Equity and Fairness which states:

i. PHARMAC must return to the negotiating table with the supplier of the Soliris treatment

ii. PHARMAC must negotiate in good faith toward funding Soliris for a minimum of 8 NZ PNH patients

iii. PHARMAC must establish fair assessment criteria, based on expert advice from the international haematological community, to assess patient need for the Soliris treatment

iv. PHARMAC must amend its Operating Policies and Procedures to acknowledge the right of rare disease patients to access life restoring and life saving treatments as in the specific example of the Soliris treatment

Yours faithfully

Bodie Etheredge
Sent from my Samsung

From:	Scott Jones [redacted]
Sent:	Wednesday, 24 July 2013 5:28 p.m.
To:	eculizumabfeedback
Subject:	submission to Pharmac.docx
Attachments:	submission to Pharmac.docx

Sue Anne Yee
Therapeutic Group Manager
PHARMAC

Email: eculizumabfeedback@pharmac.govt.nz
Fax: 04 460 4995
Post: PO Box 10 254, Wellington 6143

Subject: **Submission to PHARAMC'S proposal to decline a funding application for Soliris (eculizumab)**

To whom it may concern:

I support the PNH patients in New Zealand in their fight to gain access to the lifesaving treatment Soliris.

I DO NOT support Pharmac's proposed intent to decline this treatment. I also believe it would be a backward step for New Zealand as a whole.

New Zealand is a civilized democracy. We are part of the United Nations and pride ourselves on out human rights record. We hold our own on the world stage in every area of excellence in spite of being a small population. We have earned respect! Our public health system and our medicines purchasing agency (Pharmac), are held up as models by which other nations strive to aspire. However, on the issue of treatment for rare diseases, these other nations take the lead while we lethargically drag our heels. We are told by Pharmac that the issue is about cost and cost effectiveness, and there is no doubt that the new, innovative medicines are costly, not just to New Zealand, but to the international market. However, in spite of these high costs, other cash strapped nations appear to be meeting the challenge. This, in itself, makes our refusal to treat even more difficult to justify.

If we take the upholding of human rights seriously, we would agree that New Zealand citizens have a right to life. Hence, we have a moral obligation to ensure that everyone has access to life sustaining treatment when such treatments become available. No clinical population should ever be completely abandoned in our society. The notion that patients with rare diseases can be ignored because the costs are higher than average, is callous and inhumane. I recognize that it must be difficult at times to balance the books, but it would be morally wrong to achieve this by denying treatment to this small, select group of patients.

Patients with rare diseases are already disadvantaged because of deteriorating health and all that this entails; they should not be subjected to the added pressures of having to fight for the health benefits that most other New Zealanders take for granted.

As I see it, a far more compassionate approach would be for Pharmac to negotiate lower prices with drug companies, which incidentally is the precise expertise upon which Pharmac has built its international reputation. Come on Pharmac! Do the right thing! Take up this challenge and fund these drugs!

I do not support your intent to decline treatment for the PNH group or other patients where there are therapies for rare diseases.

Brenda Jones
Scott Jones

Electronically reviewed and approved.

From: Liddiard Laurent Brenda [redacted]
Sent: Wednesday, 19 June 2013 10:21 a.m.
To: eculizumabfeedback
Subject: Submission to PHARMAC's Proposal to decline a funding application for Soliris (eculizumab)

To Whom it May Concern;

I support PNH patients in New Zealand in their fight to gain access to the life saving treatment Soliris.

I DO NOT support PHARMAC's proposal to decline funding for the Soliris treatment.

I support the PNH Support Association's proposition for Equity and Fairness which states:

i. **PHARMAC must return to the negotiating table** with the supplier of the Soliris treatment

ii. **PHARMAC must negotiate in good faith** toward funding Soliris for a minimum of 8 NZ PNH patients

iii. **PHARMAC must establish fair assessment criteria**, based on expert advice from the international haematological community, to assess patient need for the Soliris treatment

iv. **PHARMAC must amend its Operating Policies and Procedures** to acknowledge the right of rare disease patients to access life restoring and life saving treatments as in the specific example of the Soliris treatment

The question I would ask decision-makers on this issue is, "How would you feel if one of your close family members suffered from this terrible (and treatable) disease, and was being denied medication that could potentially extend their life expectancy by several decades?". For these people, and other sufferers of rare diseases, to be told that their lives are not worth saving because treatment is too expensive is totally devastating.

Where is the justice in a situation where millions of public dollars are spent every year on medicines to treat self-inflicted, lifestyle illnesses, when sufferers of such rare diseases, such as PNH find themselves facing a death sentence through no fault of their own?

This situation should not be occurring in New Zealand. Please think again.

Yours faithfully

Brenda Liddiard Laurent

To: eculizumabfeedback[eculizumabfeedback@Pharmac.govt.nz];
Flag Status: 0x00000000
Subject: Soliris Funding
From: [redacted]
Sent: Mon 6/17/2013 7:26:56 **

17 June 2013

[redacted]

[redacted]

[redacted]

PHARMAC

TO WHOM IT MAY CONCERN:

With regards to your proposal to decline funding for Soliris.

My [redacted], [redacted], has been very unlucky and lucky in her short life.

Unlucky as by the time [redacted] was [redacted] age [redacted] was critically ill. After 9 long months of [redacted] [redacted] was diagnosed with PNH (Paroxysmal Nocturnal Heamoglobinuria).

I cannot recall how long we have spent in hospital over the past years. Watching my young [redacted] suffer has been truly heart-breaking.

After years of struggling with chronically poor health I became aware of the life-saving treatment Soliris, which offered my [redacted] and I hope.

With the enormous supportive efforts of [redacted] and after I fought on my [redacted] behalf, [redacted] is now lucky to be one of the New Zealand patients who now receive Soliris on compassionate grounds. This drug has changed [redacted] life!!

Previously [redacted]

Now, with Soliris, [redacted]'s life has been transformed. [redacted] has a job, enjoys socialising and is able to get on with [redacted] life as any young [redacted] deserves to be able to do. [redacted] can do basic things now that many of us take for granted such as join a gym, go for a long walk, participate and attend events that once were not possible, either because [redacted] was not physically able to or felt well enough to do.

The years and years of struggling, the time spent in hospital, the near fatal health complications feel more and more distant as [redacted] life moves forward.

To deny this life saving drug to New Zealanders living with PNH is unbelievable. It is like handing down a life sentence.

I urge you to do the right thing and make this LIFE SAVING drug available to those who need it!

Kind regards

[redacted]

[redacted]

To: eculizumabfeedback[eculizumabfeedback@Pharmac.govt.nz]; OPP Review[opp@Pharmac.govt.nz];
Subject: Email submission
Sent: Mon 7/29/2013 9:46:28 **
From: Staff

To Whom it May Concern;

I support PNH patients in New Zealand in their fight to gain access to the life saving treatment Soliris.

I DO NOT support PHARMAC's proposal to decline funding for the Soliris treatment.

I support the PNH Support Association's proposition for Equity and Fairness which states:
i. PHARMAC must return to the negotiating table with the supplier of the Soliris treatment
ii. PHARMAC must negotiate in good faith toward funding Soliris for a minimum of 8 NZ PNH patients
iii. PHARMAC must establish fair assessment criteria, based on expert advice from the international haematological community, to assess patient need for the Soliris treatment
iv. PHARMAC must amend its Operating Policies and Procedures to acknowledge the right of rare disease patients to access life restoring and life saving treatments as in the specific example of the Soliris treatment

Yours faithfully

Brenda Stuart
[redacted]

To: OPP Review[opp@Pharmac.govt.nz];
eculizumabfeedback[eculizumabfeedback@Pharmac.govt.nz];
Cc: [redacted]
Flag Status: 0x00000000
Subject: Pharmac's consultation on Decision Criteria
From: Bronwyn Gray
Sent: Wed 6/12/2013 3:22:39 ..

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	by smtp101.tnz.mail.aue.yahoo.com with SMTP; 11 Jun 2013 20:22:41 -0700 PDT
From: Bronwyn Gray [redacted]
To: <opp@pharmac.govt.nz>, <eculizumabfeedback@pharmac.govt.nz>
CC: [redacted]
Subject: Pharmac's consultation on Decision Criteria
Date: Wed, 12 Jun 2013 15:22:39 +1200
Message-ID: <000f01ce671c$18072630$48157290$@xtra.co.nz>
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Return-Path: les.bron.boatshed.bay@xtra.co.nz
X-MS-Exchange-Organization-AuthSource: PharmExchange.Pharmac.dom
X-MS-Exchange-Organization-AuthAs: Anonymous
X-EsetId: 7B7D663C0DCF3334283E3B
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To Whom it may Concern

PAROXYSMAL NOCTURNAL HEMOGLOBINURIA (PNH)

I write in support of PNH patients in New Zealand in their fight to gain access to the life saving treatment: (ECULIZUMAB)**SOLIRIS.**

I do not support Pharmac's proposal to decline funding for the Soliris Treatment.

I support the PNH Support Association's proposition for Equity and Fairness which states:

1) Pharmac must return to the Negotiating Table with Alexion Pharmaceuticals, the supplier of the Soliris treatment

2) Pharmac must negotiate in good faith towards funding Soliris for a minimum of 8 New Zealand PNH patients

3)Pharmac must establish fair assessment criteria, based on expert advice from the international haematological community, to assess patient need for the Soliris treatment

4) Pharmac must amend its Operating Policies and Procedures to acknowledge the right of rare disease patients to access life restoring and life saving treatments, a specific example of which is the Soliris Treatment

PNH patients along with all New Zealanders, deserve to be treated fairly and with respect, under the Universal Declaration of Human Rights (Article 25) to which New Zealand is a signatory.

In our country the right to health is expressed in a variety of legislation.

Of these laws, the most directly applicable to the right to health and to healthcare in particular, is the New Zealand Public Health and Disability Act 2000 (PHDA) which provides a structure for the delivery of personal health, public health and disability support services, with the objectives of:

*improving, promoting and protecting health

*promoting the inclusion and participation in society and the independence of disabled people

*ensuring the best care and support for those in need of services.

I submit that it is critical that Pharmac considers the plight of those living with PNH by reaching an agreement with the company that supplies Soliris, and to fund the medication for those whose lives desperately depend on it.

As a submitter, I am ashamed that New Zealand is the only OECD Country that proposes to decline funding this drug.

I am further ashamed that Pharmac's proposal to decline the funding is consistent with " the clinical advice we have received"...because although it is an effective treatment, it is extremely expensive."

Yours faithfully

Bronwyn Gray JP QSM

Director

New Zealand LAM Trust

director@lam.org.nz

www.lam.org.nz

NZORD Board member

New Zealand Organisation for Rare Disorders

www.nzord.org.nz

[redacted]

[redacted]

To: eculizumabfeedback[eculizumabfeedback@Pharmac.govt.nz];
Subject: Fwd: Submission to PHARAMC'S proposal to decline a funding application for Soliris (eculizumab)
Sent: Sun 7/28/2013 10:25:55 ..
From: Bruce Wells

To whom it may concern:

I support the PNH patients in New Zealand in their fight to gain access to the lifesaving treatment Soliris.

I DO NOT support Pharmac's proposed intent to decline this treatment. I also believe it would be a backward step for New Zealand as a whole.

New Zealand is a civilized democracy. We are part of the United Nations and pride ourselves on out human rights record. We hold our own on the world stage in every area of excellence in spite of being a small population. We have earned respect! Our public health system and our medicines purchasing agency (Pharmac), are held up as models by which other nations strive to aspire. However, on the issue of treatment for rare diseases, these other nations take the lead while we lethargically drag our heels. We are told by Pharmac that the issue is about cost and cost effectiveness, and there is no doubt that the new, innovative medicines are costly, not just to New Zealand, but to the international market. However, in spite of these high costs, other cash strapped nations appear to be meeting the challenge. This, in itself, makes our refusal to treat even more difficult to justify.

If we take the upholding of human rights seriously, we would agree that New Zealand citizens have a right to life. Hence, we have a moral obligation to ensure that everyone has access to life sustaining treatment when such treatments become available. No clinical population should ever be completely abandoned in our society. The notion that patients with rare diseases can be ignored because the costs are higher than average, is callous and inhumane. I recognize that it must be difficult at times to balance the books, but it would be morally wrong to achieve this by denying treatment to this small, select group of patients.

Patients with rare diseases are already disadvantaged because of deteriorating health and all that this entails; they should not be subjected to the added pressures of having to fight for the health benefits that most other New Zealanders take for granted.

As I see it, a far more compassionate approach would be for Pharmac to negotiate lower prices with drug companies, which incidentally is the precise expertise upon which Pharmac has built its international reputation.

I do not support your intent to decline treatment for the PNH group or other patients where there are therapies for rare diseases.

Bruce Wells

From: Cali [redacted]
Sent: Wednesday, 26 June 2013 2:27 p.m.
To: eculizumabfeedback
Cc: OPP Review
Subject: Submission to PHARMAC's Proposal to decline a funding application for Soliris (eculizumab)Subject: Submission in response to PHARMAC's consultation on Decision Critera.

To Whom it May Concern;

I support PNH patients in New Zealand in their fight to gain access to the life saving treatment Soliris.

I DO NOT support PHARMAC's proposal to decline funding for the Soliris treatment.

I support the PNH Support Association's proposition for Equity and Fairness which states:

i. **PHARMAC must return to the negotiating table** with the supplier of the Soliris treatment

ii. **PHARMAC must negotiate in good faith** toward funding Soliris for a minimum of 8 NZ PNH patients

iii. **PHARMAC must establish fair assessment criteria,** based on expert advice from the international haematological community, to assess patient need for the Soliris treatment

iv. **PHARMAC must amend its Operating Policies and Procedures** to acknowledge the right of rare disease patients to access life restoring and life saving treatments as in the specific example of the Soliris treatment

From: Cam Morgan [redacted]
Sent: Monday, 29 July 2013 12:04 p.m.
To: eculizumabfeedback; OPP Review
Subject: Submission opposing PHARMAC's Proposal to decline a funding application for Soliris (eculizumab)

Re: Submission opposing PHARMAC's Proposal to decline a funding application for Soliris (eculizumab)

To Whom it May Concern;

I SUPPORT PNH patients in New Zealand in their fight to gain access to the lifesaving treatment Soliris.

I OPPOSE PHARMAC's proposal to decline funding for the Soliris treatment.

I support the PNH Support Association's proposition for Equity and Fairness which states:

i. **PHARMAC must return to the negotiating table** with the supplier of the Soliris treatment

ii. **PHARMAC must negotiate in good faith** toward funding Soliris for a minimum of 8 NZ PNH patients

iii. **PHARMAC must establish fair assessment criteria,** based on expert advice from the international haematological community, to assess patient need for the Soliris treatment

iv. **PHARMAC must amend its Operating Policies and Procedures** to acknowledge the right of rare disease patients to access life restoring and lifesaving treatments as in the specific example of the Soliris treatment

It is imperative that people get a fair chance for reasonable medical support, no matterhow rear, these people are at great risk without your funding support.

please reconsider your stance.

Yours faithfully

Campbell morgan

Campbell Morgan | Modern Apprentice Coordinator | Trade Education Ltd |
[redacted]

Website: www.tradeeducation.co.nz

From: Casey Sommerville [redacted]
Sent: Tuesday, 30 July 2013 5:44 p.m.
To: eculizumabfeedback

To whom it may concern,

Please reconsider funding the life-saving medicine Soliris. Its so hard to know there are people here in nz that have been given a death sentence due to financial burden. If its in your power to save a person, how can you sit back and let them die? Its not fair for ones basic human right to be taken away as its too difficult or too costly.

Thank you for your time,

Casey Sommerville

Sent from Samsung Mobile

From: cassy drinkwater [redacted]
Sent: Wednesday, 5 June 2013 9:04 a.m.
To: eculizumabfeedback
Subject: Submission to PHARMAC's Proposal to decline a funding application for Soliris (eculizumab)

To Whom it May Concern;

I support PNH patients in New Zealand in their fight to gain access to the life saving treatment Soliris.

I DO NOT support PHARMAC's proposal to decline funding for the Soliris treatment.

I support the PNH Support Association's proposition for Equity and Fairness which states:

i. **PHARMAC must return to the negotiating table** with the supplier of the Soliris treatment

ii. **PHARMAC must negotiate in good faith** toward funding Soliris for a minimum of 8 NZ PNH patients

iii. **PHARMAC must establish fair assessment criteria**, based on expert advice from the international haematological community, to assess patient need for the Soliris treatment

iv. **PHARMAC must amend its Operating Policies and Procedures** to acknowledge the right of rare disease patients to access life restoring and life saving treatments as in the specific example of the Soliris treatment

Yours faithfully

Cassy Williams

From: Cecilie McShane [redacted]
Sent: Wednesday, 26 June 2013 10:54 a.m.
To: eculizumabfeedback
Subject: Medicine funding equality for all New Zealanders

To Whom it May Concern;

I support PNH patients in New Zealand in their fight to gain access to the life saving treatment Soliris.

I DO NOT support PHARMAC's proposal to decline funding for the Soliris treatment.

I support the PNH Support Association's proposition for Equity and Fairness which states:

i. **PHARMAC must return to the negotiating table** with the supplier of the Soliris treatment

ii. **PHARMAC must negotiate in good faith** toward funding Soliris for a minimum of 8 NZ PNH patients

iii. **PHARMAC must establish fair assessment criteria**, based on expert advice from the international haematological community, to assess patient need for the Soliris treatment

iv. **PHARMAC must amend its Operating Policies and Procedures** to acknowledge the right of rare disease patients to access life restoring and life saving treatments as in the specific example of the Soliris treatment

We believe in equality for all New Zealanders to have access to proper medical care and medications is apart of that regime.

Yours faithfully

Cecilie McShane

From: Kate Russell [redacted]
Sent: Tuesday, 30 July 2013 3:07 p.m.
To: eculizumabfeedback
Subject: submission from CFANZ
Attachments: Soliris_submission.pdf

Good Afternoon Please find submission attached

Kind regards.

Kate

Kate Russell FFINZ
Chief Executive

CYSTIC FIBROSIS
ASSOCIATION OF NEW ZEALAND
[redacted]

CYSTIC FIBROSIS
ASSOCIATION OF NEW ZEALAND

Submission: Proposal to decline funding application for Soliris

The following submission is made on behalf of the members of the Cystic Fibrosis Association of New Zealand. The Members of the Cystic Fibrosis Association of New Zealand, support PNH patients in New Zealand in their campaign to gain access to Soliris and will indeed support any group of patients who fight to gain access to lifesaving or sustaining treatment that, due to the current policies and priorities of Pharmac and the Ministry of Health, is denied to them.

1. It is of supreme and primary concern to the CF Association that in undertaking this consultation, Pharmac is relying on spurious figures and information to support its position. In this consultation you have overstated the number of people affected with PNH which in turn over exaggerates the likely real cost of treatment. This also happened in 2011 when Pharmac staff reported to the Board that there would be up to 100 affected patients in New Zealand with Pompe disease. And in the 2012 when Pharmac staff grossly overstated the number of Cystic Fibrosis patients in New Zealand during the consultation on Special Foods. Here we have three demonstrated and recent cases where it appears that Pharmac staff have wilfully overinflated numbers to support their case in declining treatment . This is unacceptable and does nothing to enhance or support Pharmac's reputation as a trusted or effective agent of the Crown and indeed, in our opinion, leaves the way open for a legal challenge to any decisions made on the basis of incorrect information. It is also disturbing that when these figures have been challenged on all the occasions above, Pharmac staff seem unconcerned that their information sources are so unreliable.

2. The members of the CF Association are concerned that we have a system that considers that some conditions can be treated because enough people suffer from it to allow bulk buying to be possible and others, who through no fault of their own, are born with a 'rare' condition, are considered to have lives that are expendable due to the cost of medications that cannot be bought in sufficient quantity to get cost gains. This approach is both grossly unfair and the thinking behind it, overly simplistic

3. The Members of the CF Association believe that rare and high cost conditions are not currently dealt with in a fair and equitable manner, by the policies and decision criteria applied by Pharmac. Whilst Pharmac argues that the issue of High Cost Medicines was adequately dealt with by the study done by McCormack and Hansen some years ago, in fact it does nothing to address the simple fact that if and when medications for rare conditions are placed in competition with drugs for more common conditions, they are ALWAYS doomed to fail. In the meantime, lives continue to be lost, quality of life deteriorates and people suffer. The system needs to change.

The members of the Cystic Fibrosis Association of New Zealand **do not** support the decision to decline funding for Soliris and call for Pharmac to address, with the Ministry of Health, the issues of funding for high cost medicines and rare conditions. To do anything else is to fail in the most basic duty of care our Government and her agents have to her citizens, to support their right to good health and equitable access to life-saving and quality of life enhancing drugs.

Kate Russell
Chief Executive
Cystic Fibrosis Association of New Zealand

From: [redacted]
Sent: Tuesday, 30 July 2013 8:30 p.m.
To: eculizumabfeedback
Cc: opp@pharmc.govt.nz
Subject: Submission to pharmacy proposal to decline funding for Solaris (eculizumab)

[redacted]

To Whom it May Concern;

I support PNH patients in New Zealand in their fight to gain access to the life saving treatment Soliris.

I DO NOT support PHARMAC's proposal to decline funding for the Soliris treatment.

I support the PNH Support Association's proposition for Equity and Fairness which states:

i.
PHARMAC must return to the negotiating table with the supplier of the Soliris treatment

ii.
PHARMAC must negotiate in good faith toward funding Soliris for a minimum of 8 NZ PNH patients

iii.
PHARMAC must establish fair assessment criteria, based on expert advice from the international haematological community, to assess patient need for the Soliris treatment

iv.
PHARMAC must amend its Operating Policies and Procedures to acknowledge the right of rare disease patients to access life restoring and life saving treatments as in the specific example of the Soliris treatment

As an Aunt of a young boy with a rare genetic immunity disorder who has weekly treatment to keep him fit and healthy I am appalled that funding is not available for this life saving treatment. When my nephew was on life support with little hope of surviving after catching the common cold he was given a second chance by the British Governments support by funding his treatment. all Children deserve this if there is treatment available!

Yours faithfully

[redacted]

Sent from my iPad

From: Christopher Cooke [redacted]
Sent: Sunday, 2 June 2013 10:42 p.m.
To: eculizumabfeedback
Subject: Submission to PHARMAC's Proposal to decline a funding application for Soliris (eculizumab)

To Whom it May Concern;

I support PNH patients in New Zealand in their fight to gain access to the life saving treatment Soliris.

I DO NOT support PHARMAC's proposal to decline funding for the Soliris treatment.

I support the PNH Support Association's proposition for Equity and Fairness which states:

i. **PHARMAC must return to the negotiating table** with the supplier of the Soliris treatment

ii. **PHARMAC must negotiate in good faith** toward funding Soliris for a minimum of 8 NZ PNH patients

iii. **PHARMAC must establish fair assessment criteria**, based on expert advice from the international haematological community, to assess patient need for the Soliris treatment

iv. **PHARMAC must amend its Operating Policies and Procedures** to acknowledge the right of rare disease patients to access life restoring and life saving treatments as in the specific example of the Soliris treatment

Please see the value that funding this treatment will have on the families, friends and loved ones of PNH sufferers. See the value, not the cost.

Yours faithfully

Chris Cooke

From: [redacted]
Sent: Sunday, 2 June 2013 1:43 p.m.
To: eculizumabfeedback
Subject: Proposal to decline a funding application for eculizumab.

To
Sue Anne Yee
Therapuetic Group Manager
PHARMAC
from
[redacted]
Email
[redacted]

Dear Ms Yee,
On behalf of my wife, son and daughter-in-law I would very much plead with PHARMAC to reconsider your decision not to fund eculizumab. Our [redacted] daughter-in-law was sent back to her country after having her baby in Australia because she was told that the Australian Government considered the cost of Soliris to expensive ! She contracted PNH when she became pregnant at the age of [redacted]. As parents-in-law we love our daghter-in-law and have seen her suffering her disease most stoically, and of course our son and his daughter also have to live with it every day.
With only twenty sufferers with PNH in NZ how can you genuinely say to them that the cost is to great for the NZ Government ?
On my families behalf I beg your organisation to reconsider your decision and change the result into an affirmative one for all NZ sufferers of PNH.
Yours faihfully,
[redacted]

From: Slades [redacted]
Sent: Saturday, 1 June 2013 9:38 p.m.
To: eculizumabfeedback
Subject: Submission to PHARMAC's Proposal to decline a funding application for Soliris (eculizumab)

To Whom it May Concern;

I support PNH patients in New Zealand in their fight to gain access to the life saving treatment Soliris.

I DO NOT support PHARMAC's proposal to decline funding for the Soliris treatment.

I support the PNH Support Association's proposition for Equity and Fairness which states:

i. **PHARMAC must return to the negotiating table** with the supplier of the Soliris treatment

ii. **PHARMAC must negotiate in good faith** toward funding Soliris for a minimum of 8 NZ PNH patients

iii. **PHARMAC must establish fair assessment criteria**, based on expert advice from the international haematological community, to assess patient need for the Soliris treatment

iv. **PHARMAC must amend its Operating Policies and Procedures** to acknowledge the right of rare disease patients to access life restoring and life saving treatments as in the specific example of the Soliris treatment

Basically it comes down to human rights and dignity.

It is illegal to kill people who want die when they are terminal, yet perfectly legal for us to turn our backs on those who want to live. That makes no sense. It makes no sense...

Yours faithfully

Christina Slade
[redacted]

From: David & Michelle McDonnell [redacted]
Sent: Tuesday, 23 July 2013 6:41 p.m.
To: eculizumabfeedback
Subject: Soliris

Subject: **Submission to PHARAMC'S proposal to decline a funding application for Soliris (eculizumab)**

To whom it may concern:

I support the PNH patients in New Zealand in their fight to gain access to the lifesaving treatment Soliris.

I **DO NOT** support Pharmac's proposed intent to decline this treatment.

- When Pharamc takes this type of consultation to the public I should be able to rely on Pharmac to provide accurate and reliable information that will help guide responses from the community. In this consultation you have overstated the number of people affected with PNH which in turn over exaggerates the likely real cost of treatment. You did this back in 2011 when Pharmac staff reported to the Board that there would be up to 100 affected patients in New Zealand with Pompe disease. That treatment was declined, and it looks as though you intend to decline Soliris, again with dubious data as the basis for a decision. This is not acceptable and you should withdraw this consultation because of the misleading information in it, which is likely to skew responses from the public.

- These patients have a right to life. Our health system has a duty to address their needs in a fair and equitable way. When Pharmac places so much emphasis on costs, cost-effectiveness and alternative use of the money, but does not address issues of rights, equity, fairness and community values; you are failing in your duty. The District Health Boards have this duty, and Pharmac is acting as their purchasing agency. You should use the same decision criteria and priorities that they have.

- Patients with rare diseases are disadvantaged because their condition is rare, and when treatments become available they are doubly affected by the high cost and the very small market due to small numbers. It is a denial of their right to health, and contrary to goals of "Equity of access, reducing inequalities and improving health outcomes for individuals and communities will guide our relationship and decision making", which are set out in the agreement between DHBs and Pharmac about how you decide things on their behalf.

- **Decision Criteria:** The decision criteria used to assess medicines are not fair for those who are affected by rare diseases. There should be an additional layer of decision-making for rare diseases that do not fit the standard cost effectiveness threshold for large populations. These additional layers already exist in Australia, England, Scotland and other places around the world, because they have recognised that it's a fair way to deal with the disadvantage of rare diseases.

- Pharmac's assumption that "best health outcomes" as mentioned in its legislation, can be strongly associated with calculation of Quality Adjusted Life Years, is too narrow a view of what is "best", especially when Pharmac's calculations do not take into account non-health-sector costs. The decision criteria should include a broader range of considerations that are important to patients, so that the decisions are made in a patient-centred way.

- Pharmac also has a responsibility for "health outcomes that are reasonably achievable". Your narrow perspective on technical assessment and budget management, to the exclusion of patient rights and interests from decisions, are an outcome that effectively discriminates against patients with rare diseases, and is not a reasonable outcome by any measure.

- Pharmac often emphasises the "tough decision" approach that some will be funded and others not. That is too simplistic and can also be unfair, as it is in this case. There are very good reasons to adopt an equitable approach that spreads medicine funding across as many areas of health need as possible, so that no group is completely **intent to decline treatment** for the PNH group or other patients where there are therapies for rare diseases. abandoned. That is the approach across a wide range of services in our health system and Pharmac should adopt a similar approach, rather than exclude and abandon certain groups.

I do not support your

From: David Paddock [redacted]
Sent: Sunday, 16 June 2013 4:14 p.m.
To: eculizumabfeedback; OPP Review
Subject: Submission to PHARMAC's Proposal to decline a funding

To Whom it May Concern;

I support PNH patients in New Zealand in their fight to gain access to the life saving treatment Soliris.

I DO NOT support PHARMAC's proposal to decline funding for the Soliris treatment.

I support the PNH Support Association's proposition for Equity and Fairness which states:

i. **PHARMAC must return to the negotiating table** with the supplier of the Soliris treatment

ii. **PHARMAC must negotiate in good faith** toward funding Soliris for a minimum of 8 NZ PNH patients

iii. **PHARMAC must establish fair assessment criteria,** based on expert advice from the international haematological community, to assess patient need for the Soliris treatment

iv. **PHARMAC must amend its Operating Policies and Procedures** to acknowledge the right of rare disease patients to access life restoring and life saving treatments as in the specific example of the Soliris treatment

Yours faithfully

David Paddock

From: [redacted] [redacted]
Sent: Sunday, 28 July 2013 9:22 p.m.
To: eculizumabfeedback; OPP Review
Subject: Submission to PHARMAC's Proposal to decline a funding application for Soliris (eculizumab)

To Whom it May Concern;

I support PNH patients in New Zealand in their fight to gain access to the life saving treatment Soliris.

I DO NOT support PHARMAC's proposal to decline funding for the Soliris treatment.

I support the PNH Support Association's proposition for Equity and Fairness which states:

i. **PHARMAC must return to the negotiating table** with the supplier of the Soliris treatment

ii. **PHARMAC must negotiate in good faith** toward funding Soliris for a minimum of 8 NZ PNH patients

iii. **PHARMAC must establish fair assessment criteria**, based on expert advice from the international haematological community, to assess patient need for the Soliris treatment

iv. **PHARMAC must amend its Operating Policies and Procedures** to acknowledge the right of rare disease patients to access life restoring and life saving treatments as in the specific example of the Soliris treatment

Please provide the PNH patients with a chance live by funding Soliris treatment in New Zealand. I know how patients benefitted from Soliris personally as my sister-in-law is currently leading a normal life due to Soliris. It gave her a chance to live, have hope and live a normal life.

Yours faithfully

[redacted]

From:	Dawn Jones [redacted]
Sent:	Tuesday, 30 July 2013 9:38 a.m.
To:	eculizumabfeedback
Subject:	Fw: Submission to PHARMAC'S proposal to decline a funding application for Soliris (eculizumab)

Sue Anne Yee
Therapeutic Group Manager
PHARMAC

Email: eculizumabfeedback@pharmac.govt.nz
Fax: 04 460 4995
Post: PO Box 10 254, Wellington 6143

Subject: **Submission to PHARAMC'S proposal to decline a funding application for Soliris (eculizumab)**

To whom it may concern:

I support the PNH patients in New Zealand in their fight to gain access to the lifesaving treatment Soliris.

I **DO NOT** support Pharmac's proposed intent to decline this treatment.

- When Pharmac takes this type of consultation to the public I should be able to rely on Pharmac to provide accurate and reliable information that will help guide responses from the community. In this consultation you have overstated the number of people affected with PNH which in turn over exaggerates the likely real cost of treatment. You did this back in 2011 when Pharmac staff reported to the Board that there would be up to 100 affected patients in New Zealand with Pompe disease. That treatment was declined, and it looks as though you intend to decline Soliris, again with dubious data as the basis for a decision. This is not acceptable and you should withdraw this consultation because of the misleading information in it, which is likely to skew responses from the public.

- These patients have a right to life. Our health system has a duty to address their needs in a fair and equitable way. When Pharmac places so much emphasis on costs, cost-effectiveness and alternative use of the money, but does not address issues of rights, equity, fairness and community values; you are failing in your duty. The District Health Boards have this duty, and Pharmac is acting as their purchasing agency. You should use the same decision criteria and priorities that they have.

- Patients with rare diseases are disadvantaged because their condition is rare, and when treatments become available they are doubly affected by the high cost and the very small market due to small numbers. It is a denial of their right to health, and contrary to goals of "Equity of access, reducing inequalities and improving health outcomes for individuals and communities will guide our relationship and decision making", which are set out in the agreement between DHBs and Pharmac about how you decide things on their behalf.

- **Decision Criteria: The decision criteria used to assess medicines are not fair for those who are affected by rare diseases. There should be an additional layer of decision-making for rare diseases that do not fit the standard cost effectiveness threshold for large populations. These additional layers already exist in Australia, England, Scotland and other places around the**

world, because they have recognised that it's a fair way to deal with the disadvantage of rare diseases.

- Pharmac's assumption that "best health outcomes" as mentioned in its legislation, can be strongly associated with calculation of Quality Adjusted Life Years, is too narrow a view of what is "best", especially when Pharmac's calculations do not take into account non-health-sector costs. The decision criteria should include a broader range of considerations that are important to patients, so that the decisions are made in a patient-centred way.

- Pharmac also has a responsibility for "health outcomes that are reasonably achievable". Your narrow perspective on technical assessment and budget management, to the exclusion of patient rights and interests from decisions, are an outcome that effectively discriminates against patients with rare diseases, and is not a reasonable outcome by any measure.

- Pharmac often emphasises the "tough decision" approach that some will be funded and others not. That is too simplistic and can also be unfair, as it is in this case. There are very good reasons to adopt an equitable approach that spreads medicine funding across as many areas of health need as possible, so that no group is completely abandoned. That is the approach across a wide range of services in our health system and Pharmac should adopt a similar approach, rather than exclude and abandon certain groups.

I do not support your intent to decline treatment for the PNH group or other patients where there are therapies for rare diseases.

From: [redacted]
Sent: Wednesday, 31 July 2013 6:44 p.m.
To: eculizumabfeedback
Subject: medicine

Pharmac ...

We have educated our best and brightest for years to discover treatments for diseases that only 30yrs ago would have been marvelled at.

A way has to be found to manage the economics so that all people needing these life saving drugs can have them ..

- **Decision Criteria:** The decision criteria used to assess medicines are not fair for those who are affected by rare diseases. There should be an additional layer of decision-making for rare diseases that do not fit the standard cost effectiveness threshold for large populations. These additional layers already exist in Australia, England, Scotland and other places around the world, because they have recognised that it's a fair way to deal with the disadvantage of rare diseases.

- Pharmac's assumption that "best health outcomes" as mentioned in its legislation, can be strongly associated with calculation of Quality Adjusted Life Years, is too narrow a view of what is "best", especially when Pharmac's calculations do not take into account non-health-sector costs. The decision criteria should include a broader range of considerations that are important to patients, so that the decisions are made in a patient-centred way.

- Pharmac also has a responsibility for "health outcomes that are reasonably achievable". Your narrow perspective on technical assessment and budget management, to the exclusion of patient rights and interests from decisions, are an outcome that effectively discriminates against patients with rare diseases, and is not a reasonable outcome by any measure.

- Pharmac often emphasises the "tough decision" approach that some will be funded and others not. That is too simplistic and can also be unfair, as it is in this case. There are very good reasons to adopt an equitable approach that spreads medicine funding across as many areas of health need as possible, so that no group is completely abandoned. That is the approach across a wide range of services in our health system and Pharmac should adopt a similar approach, rather than exclude and abandon certain groups.

I do not support your intent to decline treatment for the PNH group or other patients where there are therapies for rare diseases.

To know these drugs are available but are denied you due to budget restraints, is cruel.

Employ the wonderful economic intellects and managers we now have to find away, we educated them for this purpose too.

Sincerely,

Diane Bellamy

When the power of love overcomes the love of power, the world will know peace.

From: Dion Mehring [redacted]
Sent: Tuesday, 4 June 2013 10:08 p.m.
To: eculizumabfeedback
Subject: Submission to PHARMAC's Proposal to decline a funding application for Soliris (eculizumab)

To Whom it May Concern;

I support PNH patients in New Zealand in their fight to gain access to the life saving treatment Soliris.

I DO NOT support PHARMAC's proposal to decline funding for the Soliris treatment.

I support the PNH Support Association's proposition for Equity and Fairness which states:

i. **PHARMAC must return to the negotiating table** with the supplier of the Soliris treatment

ii. **PHARMAC must negotiate in good faith** toward funding Soliris for a minimum of 8 NZ PNH patients

iii. **PHARMAC must establish fair assessment criteria**, based on expert advice from the international haematological community, to assess patient need for the Soliris treatment

iv. **PHARMAC must amend its Operating Policies and Procedures** to acknowledge the right of rare disease patients to access life restoring and life saving treatments as in the specific example of the Soliris treatment

(please include your personal statements here)

Yours faithfully

Jasmine Webster

From:	[redacted]
Sent:	Friday, 19 July 2013 3:16 p.m.
To:	eculizumabfeedback
Subject:	Submission to PHARMAC's Proposal to decline a funding application for Soliris (eculizumab)
Importance:	High

To Whom it May Concern,

I support PNH patients in New Zealand in their fight to gain access to the life saving treatment Soliris.

I DO NOT support PHARMAC's proposal to decline funding for the Soliris treatment.

I support the PNH Support Association's proposition for Equity and Fairness which states:

i. **PHARMAC must return to the negotiating table** with the supplier of the Soliris treatment

ii. **PHARMAC must negotiate in good faith** toward funding Soliris for a minimum of 8 NZ PNH patients

iii. **PHARMAC must establish fair assessment criteria**, based on expert advice from the international haematological community, to assess patient need for the Soliris treatment

iv. **PHARMAC must amend its Operating Policies and Procedures** to acknowledge the right of rare disease patients to access life restoring and life saving treatments as in the specific example of the Soliris treatment

My wife has been receiving Soliris treatment since it was approved in Australia and it has made a positive and major impact to her life. [redacted]

Since starting treatment she no longer suffers from any pain and has energy to carry out everyday activities. She is now able to live a normal life. Soliris is an amazing drug that has saved my wife's life. I urge you to support funding of Soliris to end the suffering of the NZ PNH patients.

Yours faithfully,

[redacted]

From: Dominique Cooper [redacted]
Sent: Friday, 31 May 2013 9:48 p.m.
To: eculizumabfeedback
Subject: Submissions in response to PHARMAC's Proposal to Decline Funding for Soliris (eculizumab)

Friday, 31st May 2013

To Whom it May Concern;

I support PNH patients in New Zealand in their fight to gain access to the life saving treatment Soliris.

I DO NOT support PHARMAC's proposal to decline funding for the Soliris treatment.

I support the PNH Support Association's proposition for Equity and Fairness which states:

i. PHARMAC must return to the negotiating table with the supplier of the Soliris treatment

ii. PHARMAC must negotiate in good faith toward funding Soliris for a minimum of 8 NZ PNH patients

iii. PHARMAC must establish fair assessment criteria, based on expert advice from the international haematological community, to assess patient need for the Soliris treatment

iv. PHARMAC must amend its Operating Policies and Procedures to acknowledge the right of rare disease patients to access life restoring and life saving treatments as in the specific example of the Soliris treatment

Yours faithfully

Dominique Cooper

Sent from my iPhone

From: Donna McKenzie [redacted]
Sent: Friday, 7 June 2013 9:58 a.m.
To: eculizumabfeedback
Subject: Submission to PHARMAC's Proposal to decline a funding application for Soliris (eculizumab)

To Whom it May Concern;

I support PNH patients in New Zealand in their fight to gain access to the life saving treatment Soliris.

I DO NOT support PHARMAC's proposal to decline funding for the Soliris treatment.

I support the PNH Support Association's proposition for Equity and Fairness which states:

i. **PHARMAC must return to the negotiating table** with the supplier of the Soliris treatment

ii. **PHARMAC must negotiate in good faith** toward funding Soliris for a minimum of 8 NZ PNH patients

iii. **PHARMAC must establish fair assessment criteria**, based on expert advice from the international haematological community, to assess patient need for the Soliris treatment

iv. **PHARMAC must amend its Operating Policies and Procedures** to acknowledge the right of rare disease patients to access life restoring and life saving treatments as in the specific example of the Soliris treatment

Yours faithfully

Donna McKenzie

[redacted]

[redacted]

[redacted]

From: [redacted] [redacted]
Sent: Tuesday, 23 July 2013 9:09 p.m.
To: eculizumabfeedback
Subject: Submission to PHARMAC's Proposal to decline a funding application for Soliris (eculizumab)

Importance: High

To whom is may concern

I support PNH patients in New Zealand in their fight to gain access to the life saving treatment Soliris.

PLEASE Pick up negotiations with the supplier of the Soliris treatment

PLEASE Be mindful that for those who suffer from this disease there is no alternative available for them

My sister has this condition, she now relies on steroids and frequent blood transfusions to keep her going. She needs this treatment!!

I know this is a big cost and the decision is in your hands.

I plead with you to please: Get all the facts
Negotiate the best deal
Make it happen for these people – they cannot survive without this treatment

Yours faithfully

[redacted]

From: Elizabeth Hunt [redacted]
Sent: Monday, 29 July 2013 9:38 a.m.
To: eculizumabfeedback
Subject: RE: Petition on declining Soliris to patients with PNH
Attachments: Petition to Pharmac - Soliris.pdf

Attention:
Sue Anne Yee

Please find document attached, thank you.

--
Elizabeth Hunt
[redacted]

[redacted]

[redacted]

Submission to PHARMAC'S proposal to decline a funding application for Soliris (eculizumab)

Attention: Sue Anne Yee
Therapeutic Group Manager
PHARMAC

Dear Sue
I support the PNH patients in New Zealand in their fight to gain access to the lifesaving treatment Soliris.

I DO NOT support Pharmac's proposed intent to decline this treatment.

- When Pharmac takes this type of consultation to the public I should be able to rely on Pharmac to provide accurate and reliable information that will help guide responses from the community. In this consultation you have overstated the number of people affected with PNH which in turn over exaggerates the likely real cost of treatment. You did this back in 2011 when Pharmac staff reported to the Board that there would be up to 100 affected patients in New Zealand with Pompe disease. That treatment was declined, and it looks as though you intend to decline Soliris, again with dubious data as the basis for a decision. **This is not acceptable** and you should withdraw this consultation because of the misleading information in it, which is likely to skew responses from the public.

- These patients have a right to life. Our health system has a duty to address their needs in a fair and equitable way. When Pharmac places so much emphasis on costs, cost-effectiveness and alternative use of the money, but does not address issues of rights, equity, fairness and community values; you are failing in your duty. The District Health Boards have this duty, and Pharmac is acting as their purchasing agency. You should use the same decision criteria and priorities that they have.

- Patients with rare diseases are disadvantaged because their condition is rare, and when treatments become available they are doubly affected by the high cost and the very small market due to small numbers. It is a denial of their right to health, and contrary to goals of "Equity of access, reducing inequalities and improving health outcomes for individuals and communities will guide our relationship and decision making", which are set out in the agreement between DHBs and Pharmac about how you decide things on their behalf.

- **Decision Criteria:** The decision criteria used to assess medicines are not fair for those who are affected by rare diseases. There should be an additional layer of decision-making for rare diseases that do not fit the standard cost effectiveness threshold for large populations. These additional layers already exist in Australia, England, Scotland and other places around the world, because they have recognised that it's a fair way to deal with the disadvantage of rare diseases.

- Pharmac's assumption that "best health outcomes" as mentioned in its legislation, can be strongly associated with calculation of Quality Adjusted Life Years, is too narrow a view of what is "best", especially when Pharmac's calculations do not take into account non-health-sector costs. The decision criteria should include a broader range of considerations that are important to patients, so that the decisions are made in a patient-centred way.

- Pharmac also has a responsibility for "health outcomes that are reasonably achievable".

Your narrow perspective on technical assessment and budget management, to the exclusion of patient rights and interests from decisions, are an outcome that **effectively discriminates against patients with rare diseases**, and is not a reasonable outcome by any measure.

- Pharmac often emphasises the "tough decision" approach that some will be funded and others not. That is too simplistic and can also be unfair, as it is in this case. There are very good reasons to adopt an equitable approach that spreads medicine funding across as many areas of health need as possible, so that no group is completely abandoned. That is the approach across a wide range of services in our health system and Pharmac should adopt a similar approach, rather than exclude and abandon certain groups.

As someone representing a family who also has a rare disease, **I do not support your intent to decline treatment** for the PNH group or other patients where there are therapies for rare diseases.

Elizabeth Hunt
[redacted]

[redacted]

[redacted]

From: liz marshall [redacted]
Sent: Thursday, 18 July 2013 5:35 p.m.
To: eculizumabfeedback
Subject: Submission opposing PHARMAC's Proposal to decline a funding application for Soliris (eculizumab)

To Whom it May Concern;

I SUPPORT PNH patients in New Zealand in their fight to gain access to the lifesaving treatment Soliris.

I OPPOSE PHARMAC's proposal to decline funding for the Soliris treatment.

I support the PNH Support Association's proposition for Equity and Fairness which states:

i. **PHARMAC must return to the negotiating table** with the supplier of the Soliris treatment

ii. **PHARMAC must negotiate in good faith** toward funding Soliris for a minimum of 8 NZ PNH patients

iii. **PHARMAC must establish fair assessment criteria**, based on expert advice from the international haematological community, to assess patient need for the Soliris treatment

iv. **PHARMAC must amend its Operating Policies and Procedures** to acknowledge the right of rare disease patients to access life restoring and lifesaving treatments as in the specific example of the Soliris treatment

Yours faithfully

Elizabeth Marshall

From: [redacted] [redacted]
Sent: Wednesday, 12 June 2013 4:23 a.m.
To: eculizumabfeedback
Subject: Submission to PHARMAC's Proposal to decline a funding application for Soliris (eculizumab)

To Whom it May Concern;

I support PNH patients in New Zealand in their fight to gain access to the life saving treatment Soliris.

I DO NOT support PHARMAC's proposal to decline funding for the Soliris treatment.

I support the PNH Support Association's proposition for Equity and Fairness which states:

i. PHARMAC must return to the negotiating table with the supplier of the Soliris treatment

ii. PHARMAC must negotiate in good faith toward funding Soliris for a minimum of 8 NZ PNH patients

iii. PHARMAC must establish fair assessment criteria, based on expert advice from the international haematological community, to assess patient need for the Soliris treatment

iv. PHARMAC must amend its Operating Policies and Procedures to acknowledge the right of rare disease patients to access life restoring and life saving treatments as in the specific example of the Soliris treatment

One of my closest friends suffers from PNH and needs access to Soliris for the rest of her life. She is a New Zealander working in [redacted] and would not be able to return home without access to treatment.

Yours faithfully

[redacted]

From: eva farrand [redacted]
Sent: Wednesday, 31 July 2013 7:32 a.m.
To: eculizumabfeedback
Subject: Submission to PHARMAC's Proposal to decline a funding application for Soliris (eculizumab)

To Whom it May Concern;

I support PNH patients in New Zealand in their fight to gain access to the life saving treatment Soliris.

I DO NOT support PHARMAC's proposal to decline funding for the Soliris treatment.

I support the PNH Support Association's proposition for Equity and Fairness which states:

i. **PHARMAC must return to the negotiating table** with the supplier of the Soliris treatment

ii. **PHARMAC must negotiate in good faith** toward funding Soliris for a minimum of 8 NZ PNH patients

iii. **PHARMAC must establish fair assessment criteria**, based on expert advice from the international haematological community, to assess patient need for the Soliris treatment

iv. **PHARMAC must amend its Operating Policies and Procedures** to acknowledge the right of rare disease patients to access life restoring and life saving treatments as in the specific example of the Soliris treatment

Yours faithfully,

Eva Marie Farrand

From: President FSGA [redacted]
Sent: Wednesday, 24 July 2013 11:34 a.m.
To: eculizumabfeedback
Cc: Anne Hunter
Subject: Fabry Support Group Australia's Letter to PHARMAC Re proposal to decline a funding application for Soliris (eculizumab)
Attachments: FSGA Letter to PHARMAC.pdf

Dear Sue Anne Yee,

RE: Submission to PHARMAC's proposal to decline a funding application for Soliris® (eculizumab)

Please find attached a letter from the Fabry Support Group of Australia Inc. with regard to PHARMAC's proposal to decline a funding application for Soliris®.
A paper version will be posted to you for your file.

I welcome an opportunity to discuss this further either face to face whilst I am in NZ next week or over the phone.

Kindest Regards,

Megan Fookes
Director
Fabry Support Group Australia Inc.
[redacted]

This transmission is intended for the named recipient only. It may contain private and confidential information. If this comes to you in error you must not act on anything disclosed in it, nor must you copy it, modify it, disseminate it any way, or show it to anyone. Please email the sender to inform use of the transmission error or telephone Fabry Support Group Australia Inc. immediately and delete the email from your information system.

Fabry support group Australia

Sue Anne Yee
Therapeutic Group Manager
PHARMAC
Post: PO Box 10 254, Wellington 6143

24th July 2013

Dear Sue Yee,

RE: Submission to PHARMAC's proposal to decline a funding application for Soliris® ® (eculizumab)

I am writing to you as Director of the Fabry Support Group of Australia (FSGA) which represents families of those affected by Fabry Disease; a rare, genetic and often fatal lysosomal storage disorder. The Australian Federal Government provides access to funded therapies for this very rare condition to over 100 Fabry affected men and women and has done so since 2004 via a program called the Life Saving Drugs Program (LSDP). I would like to express my grave concern in PHARMAC's intention to decline a funding application for Soliris® a vital treatment needed by those living with Paroxysmal nocturnal hemoglobinuria (PNH).

In Australia the independent advisory body, Pharmaceutical Benefits Advisory Committee (PBAC) concluded that Eculizumab is an effective treatment for those living with PNH. PNH is a very rare and life-threatening blood disorder that causes premature red blood cell death. Its effects can include blood clots, kidney failure and other serious complications. Soliris® reduces the breakdown of red blood cells, the underlying cause of these complications, and therefore improves symptoms and reduces the risk of premature death or severe illness associated with PNH. Further information please read PBAC recommendation to the Australian Government dated 29 September 2010:
http://www.health.gov.au/internet/ministers/publishing.nsf/Content/BDA26C2952B5DCA2CA2577AD0000671A/$File/nr153.pdf

For any person who is born with a rare, progressive condition, one would believe that it is a basic human right to access quality care and treatment. It is simply inhumane and therefore ethically unfair to withhold vital treatment for patients with PNH. This is especially true since the appraisal process by which your neighbouring country's independent Advisory Committee; PBAC came to its recommendation was one that was comprehensive and robust; a process that is widely lauded by academia, patients and industry alike. Eculizumab is currently available to patients with Paroxysmal Nocturnal Hemoglobinuria (PNH) in Australia and many other regions globally.

Patients with rare diseases will look at this proposal and be dismayed at what they will perceive as the undermining of a trusted and fair process on purely financial grounds. It also has the potential to undermine patient confidence.

I urge you to reconsider your proposal to decline a funding application which will in turn prevent the effective treatment for those with PNH in your country. I will be in Wellington NZ on 31st July until 2nd August and would be more than happy to discuss this further with you in person or over the phone.

Kindest Regards,

Megan Fookes
Director
Fabry Support Group Australia Inc.
[redacted]

From: Faith Tapsell [redacted]
Sent: Wednesday, 31 July 2013 8:30 p.m.
To: eculizumabfeedback
Subject: Submission to PHARMAC's Proposal to decline a funding application for Soliris (eculizumab)

To Whom it May Concern;
I support PNH patients in New Zealand in their fight to gain access to the life saving treatment Soliris.
I DO NOT support PHARMAC's proposal to decline funding for the Soliris treatment.
I support the PNH Support Association's proposition for Equity and Fairness which states:
i.
PHARMAC must return to the negotiating table with the supplier of the Soliris treatment
ii.
PHARMAC must negotiate in good faith toward funding Soliris for a minimum of 8 NZ PNH patients
iii.
PHARMAC must establish fair assessment criteria, based on expert advice from the international haematological community, to assess patient need for the Soliris treatment
iv.
PHARMAC must amend its Operating Policies and Procedures to acknowledge the right of rare disease patients to access life restoring and life saving treatments as in the specific example of the Soliris treatment

Yours faithfully
Faith Tapsell

From:	Fay Te Whare [redacted]
Sent:	Friday, 19 July 2013 1:02 p.m.
To:	eculizumabfeedback
Subject:	LDNZ
Attachments:	proposition_equity_and_fairness_-_email_submission_template.doc

Please email *BOTH*

Subject: Submission to PHARMAC's Proposal to decline a funding application for Soliris (eculizumab)
Email: eculizumabfeedback@pharmac.govt.nz

AND

Subject: Submission in response to PHARMAC's consultation on Decision Critera.
Email: opp@pharmac.govt.nz

To Whom it May Concern;

I support PNH patients in New Zealand in their fight to gain access to the life saving treatment Soliris.

I DO NOT support PHARMAC's proposal to decline funding for the Soliris treatment.

I support the PNH Support Association's proposition for Equity and Fairness which states:

i. **PHARMAC must return to the negotiating table** with the supplier of the Soliris treatment

ii. **PHARMAC must negotiate in good faith** toward funding Soliris for a minimum of 8 NZ PNH patients

iii. **PHARMAC must establish fair assessment criteria**, based on expert advice from the international haematological community, to assess patient need for the Soliris treatment

iv. **PHARMAC must amend its Operating Policies and Procedures** to acknowledge the right of rare disease patients to access life restoring and life saving treatments as in the specific example of the Soliris treatment

(please include your personal statements here)To whom it may concern

It is essential that Pharmac return to the negotiating table and have informed and educative debate in regard toward funding Solaris for the 8 NZ patients affected by this rare disease. The rights of these patients is paramount in order that they may have access to life restoring and life saving treatment. These patients have a fundamental human right to achieve the goals, to work, to study and be contributing members to their families, and community. To deny them this basic right is inhuman and unethical.

We look forward to a positive response.

Yours faithfully,

Fay Te Whare.

Yours faithfully

From: Barbara Holland [redacted]
Sent: Wednesday, 31 July 2013 1:45 p.m.
To: eculizumabfeedback
Cc: [redacted]
Subject: submission
Attachments: 130731 PHARMAC endorsement decline funding for eculizumab.doc

Dear Sue Anne Yee

Please find attached a submission from the Federation of Women's Health Councils Aotearoa in support of the PHARMAC decision to decline funding of eculizumab for patients with PNH.

Yours sincerely

Barbara Holland & Barbara Robson
FWHC Co-Convenors

[redacted]

Co-Convenors:
Barbara Holland Barbara Robson
[redacted] [redacted]

FEDERATION OF
WOMEN'S HEALTH
COUNCILS
AOTEAROA • NEW ZEALAND

31 July 2013

Sue Anne Yee
Therapeutic Group Manager
PHARMAC
PO Box 10254
Wellington 6143

eculizumabfeedback@pharmac.govt.nz

Submission: Decision to decline funding of eculizumab for treatment of patients with paroxysmal nocturnal hemoglobinuria (PNH).

- FWHC **supports** the PHARMAC decision to decline funding of eculizumab (Soliris) for patients with PNH.

Note our previous correspondence 4 February 2013. We have also taken into account the considerations and recommendation of the Haematology sub-committee.

We do acknowledge it is reported to provide some treatment benefits over current treatment options; also that length of survival rates are extended for some (but not all) patients treated with this drug.

FWHC cannot find any new evidence that supports the PNHSANZ continued publicly stated claims that "this is a life-saving treatment," that it "returns life expectancy to expected norms" nor, that it is "a breakthrough for medical science". Eculizumab has not been shown to operate by a new route and is not a cure for PNH.

Of the various trials using Soliris for PNH treatments as listed on the ClinicalTrials.gov website that have been notified as being completed there are no new postings of study results. We continue to emphasise that, without the opportunity for independent scientific peer-review critique of all the findings, reliance on the evidence provided by the drug company of efficacy, safety, and long term benefit must be treated with caution. We note key authors named on the published papers publicly available to date have all declared a conflict-of-interest association with Alexion Pharmaceuticals.

- We continue to endorse the current decision criteria used by PHARMAC.

We agree that budgetary impact is a valid and significant matter to consider alongside other criteria. We do not find the equity and fairness claims for this particular patient group outweigh those of any other claimant group seeking access to new drugs.

We wish to restate our oft-repeated concerns about the growing potential for pharmaceutical companies to use an unbalanced playing field to 'educate' both patient groups/public about solutions/treatment options to 'fix the problem' through using their particular drug offering, and then purport to have brought an informed public alongside them. The public can hardly claim to be impartial advocate/decision-makers in this instance. The small PNH patient group understandably is looking for better treatment options to improve their lives and we empathise with that. They should not unnecessarily become pawns trapped in the exorbitant profit-oriented marketing games of Alexion Pharmaceuticals Ltd.

Yours sincerely

Ba Holland

Barbara Holland & Barbara Robson
Co-Convenors, FWHC

From: OPP Review
Sent: Thursday, 27 June 2013 3:37 p.m.
To: eculizumabfeedback
Subject: FW: Save lives...

From: Fialupe Lotoala [redacted]
Sent: Wednesday, 19 June 2013 10:12 p.m.
To: OPP Review
Subject: Save lives...

PHARMAC it is **unacceptable** to let people die when a life saving treatment is available.

Sent from my iPad

From: Chris Hollis [redacted]
Sent: Tuesday, 30 July 2013 10:12 p.m.
To: eculizumabfeedback
Subject: Submission to PHARMAC's proposal to decline a funding application for Soliris
Attachments: Submission to Pharmac from FXNZ - July 2013.pdf

Dear Sue Ann Yee,

Please find attached our submission on this decision.

Best regards,

Chris Hollis
Chairperson, Fragile X New Zealand

Fragile X New Zealand Trust
[redacted]

30 July 2013

Sue Anne Yee
Therapeutic Group Manager
PHARMAC

Email: eculizumabfeedback@pharmac.govt.nz
Fax: 04 460 4995
Post: PO Box 10 254, Wellington 6143

Subject: **Submission to PHARMAC's proposal to decline a funding application for Soliris (eculizumab)**

We support the PNH patients in New Zealand in their fight to gain access to the lifesaving treatment Soliris.

We DO NOT support Pharmac's proposed intent to decline this treatment

- When Pharmac take this type of consultation to the public, we should be able to rely on Pharmac to provide accurate and reliable information that will help guide responses from the community. In this consultation you have overstated the number of people affected with PNH which in turn exaggerates the likely real cost of treatment. You did this back in 2011 when Pharmac staff reported to the Board that there would be up to 100 affected patients in New Zealand with Pompe disease. That treatment was declined, and it looks as though you intend to decline Soliris, again with dubious data as the basis for a decision. This is not acceptable and you should withdraw this consultation because of the misleading information in it, which is likely to skew responses from the public.

- These patients have a right to life. Our health system has a duty to address their needs in a fair and equitable way. When Pharmac places so much emphasis on costs, cost-effectiveness and alternative use of the money, but does not address issues of rights, equity, fairness and community values, you are failing in your duty. The District Health Boards have this duty, and Pharmac is acting as their purchasing agency. You should use the same decision criteria and priorities that they have.

- Patients with rare diseases are disadvantaged because their condition is rare, and when treatments become available they are doubly affected by the high cost and the very small market due to small numbers. It is a denial of their right to health, and contrary to goals of "Equity of access, reducing inequalities and improving health outcomes for individuals and communities will guide our relationship and decision making", which are set out in the agreement between DHBs and Pharmac about how you decide things on their behalf.

- **Decision Criteria:** The decision criteria used to assess medicines are not fair for those who are affected by rare diseases. There should be an additional layer of decision-making for rare diseases that do not fit the standard cost effectiveness threshold for large populations. These additional layers already exist in Australia, England, Scotland and other places around the world, because they have recognised that it's a fair way to deal with the disadvantage of rare diseases.

- Pharmac's assumption that "best health outcomes" as mentioned in its legislation, can be strongly associated with calculation of Quality Adjusted Life Years, is too narrow a view of what is "best", especially when Pharmac's calculations do not take into account non-health-sector costs. The decision criteria should include a broader range of considerations that are important to patients, so that the decisions are made in a patient-centred way.
- Pharmac also has a responsibility for "health outcomes that are reasonably achievable". Your narrow perspective on technical assessment and budget management, to the exclusion of patient rights and interests from decisions, are an outcome that effectively discriminates against patients with rare diseases, and is not a reasonable outcome by any measure.
- Pharmac often emphasises the "tough decision" approach that some will be funded and others not. That is too simplistic and can also be unfair, as it is in this case. There are very good reasons to adopt an equitable approach that spreads medicine funding across as many areas of health need as possible so that no group is completely abandoned. That is the approach across a wide range of services in our health system and Pharmac should adopt a similar approach, rather than exclude and abandon certain groups.
- **<u>We do not support</u>** your intent to decline treatment for this group or other patients where there are therapies for rare diseases.

Yours Faithfully

Dr Christopher John Hollis, Chairperson, Fragile X New Zealand

On behalf of the trustees of the Fragile X New Zealand Trust

From: [redacted]
Sent: Tuesday, 30 July 2013 9:57 p.m.
To: eculizumabfeedback
Subject: Re:Submission on PHARMAC's proposal to decline a funding application for eculizumab

To whom it may concern,

My name is [redacted]. I am a New Zealander and the younger sister of [redacted] who has been suffering from the acquired disease PNH for as long as I can remember. Having a sister who was afflicted with the terrible burden of PNH, it has been heart breaking to witness her struggle to cope with this disease which no medical professional could offer any assistance with to allow her a normal quality of life.

[redacted] accomplished highs of life have all too often been interrupted by the unpredictable lows that are wrought by living with PNH. Despite this she has always been determined to achieve all she can as a [redacted]. She has been so stoic in her suffering, resigned to the fact that there is nothing to do but " get on with it", working her way up [redacted] in between blood transfusions and the daily symptoms of her disease.

To now know that a treatment is a possibility in New Zealand which would alleviate the symptoms of [redacted] illness and give her the chance to live her life to the fullest is incredible for me who has watched someone I love and admire be undercut by this affliction.

To be faced with the possibility of [redacted] not being able to return to the country of her birth and a society and culture which she has the capability to significantly contribute to makes me question if New Zealand is the country where I myself want to live and work.

I have lived in [redacted] for 8 years. [redacted]. I am in the process of making plans to return to NZ for good as I imagine so many New Zealanders' do after spending time in foreign pastures. I had hoped to bring back my skills to utilise in my homeland. I would never have imagined that my homeland would deny its citizens the right to life. I can see how short sighted decisions such as this would deter the "brain drain" from returning to NZ. To say I am disappointed is an understatement.

To now know that [redacted], may not ever be able to return to our mother, family and friends to be a sister, an aunt and a mother herself, because of a bureaucratic funding decision is inconceivable.

Please consider all the lives which your decisions detrimentally affect including that of my sister and my family. At the very least the criteria you use to assess such a decision should be without reproach and based on the correct facts and assessed by the appropriate experts in the field of PNH. Rare diseases deserve equal consideration to all others. In light of the fact that these are rare diseases, the drugs to treat them are going to be expensive. This factor needs to be managed rather than used as a justification to deny access to these life saving treatments.

Sincerely,

[redacted]

[redacted]

[redacted]

[redacted]

From: Gayatri Abeydeera [redacted]
Sent: Saturday, 27 July 2013 6:01 p.m.
To: eculizumabfeedback; OPP Review
Subject: Submission to PHARMAC's Proposal to decline a funding application for Soliris (eculizumab)

To Whom it May Concern;

I support PNH patients in New Zealand in their fight to gain access to the life saving treatment Soliris.

I DO NOT support PHARMAC's proposal to decline funding for the Soliris treatment.

I support the PNH Support Association's proposition for Equity and Fairness which states:

i. **PHARMAC must return to the negotiating table** with the supplier of the Soliris treatment

ii. **PHARMAC must negotiate in good faith** toward funding Soliris for a minimum of 8 NZ PNH patients

iii. **PHARMAC must establish fair assessment criteria,** based on expert advice from the international haematological community, to assess patient need for the Soliris treatment

iv. **PHARMAC must amend its Operating Policies and Procedures** to acknowledge the right of rare disease patients to access life restoring and life saving treatments as in the specific example of the Soliris treatment

Please provide the PNH patients with a chance live by funding Soliris treatment. I know how patients benefitted from Soliris personally. It gave them a chance to live, have hope and live a normal life.

Yours faithfully

Gayatri Abeydeera

[redacted]

Australia

From: geraldine mchaffie [redacted]
Sent: Wednesday, 26 June 2013 8:22 p.m.
To: eculizumabfeedback
Subject: Submission to PHARMAC's Proposal to decline a funding application for Soliris (eculizumab)

To Whom it May Concern;

I support PNH patients in New Zealand in their fight to gain access to the life saving treatment Soliris.

I DO NOT support PHARMAC's proposal to decline funding for the Soliris treatment.

I support the PNH Support Association's proposition for Equity and Fairness which states:

i. **PHARMAC must return to the negotiating table** with the supplier of the Soliris treatment

ii. **PHARMAC must negotiate in good faith** toward funding Soliris for a minimum of 8 NZ PNH patients

iii. **PHARMAC must establish fair assessment criteria**, based on expert advice from the international haematological community, to assess patient need for the Soliris treatment

iv. **PHARMAC must amend its Operating Policies and Procedures** to acknowledge the right of rare disease patients to access life restoring and life saving treatments as in the specific example of the Soliris treatment

Every life in New Zealand is worthy of saving, and Soliris is a proven means of doing so for patients with PNH.

Why can diabetics and suffers of heart disease caused by their own poor lifestyle choice receive funded treatment, yet people burdened with a genetic disorder that they have no control over cannot?

Refusing to fund this treatment which would save lives is unfair, unethical and discriminatory.

Yours faithfully

Geraldine McHaffie
[redacted]

From: [redacted]
Sent: Wednesday, 31 July 2013 10:26 p.m.
To: eculizumabfeedback
Subject: Submission
Attachments: To Whom It May Concern.doc

Please find attached my submission

Kind Regards

[redacted]

To Whom It May Concern,

I am writing to express my concern about Pharmac not funding the medication eculizumab for the PNH sufferers. My partner [redacted] suffers from PNH and although she isn't as severe as some of the other cases at this stage, I want to be able to have a future with her being happy and healthy and if she needs this medication at a later stage, I would want her to be able to access it.

I realise that this medication costs a lot of money, but how can you put a price on the life of a person just because they have a disease that is rare? It is not just my [redacted] that is suffering and if this letter goes a little way in helping change the decision of the company, I really hope it does.

Please think about this and how it can help a handful of people live a better quality of life.

Yours sincerely

[redacted]

From: Hardy, Gil [redacted]
Sent: Wednesday, 31 July 2013 8:02 p.m.
To: eculizumabfeedback
Subject: Submission to PHARMAC'S proposal to decline a funding application for Soliris (eculizumab)

To whom it may concern:

I support the PNH patients in New Zealand in their fight to gain access to the lifesaving treatment Soliris.

I **DO NOT** support Pharmac's proposed intent to decline this treatment

- When Pharmac take this type of consultation to the public I should be able to rely on Pharmac to provide accurate and reliable information that will help guide responses from the community. In this consultation you have overstated the number of people affected with PNH which in turn over exaggerates the likely real cost of treatment. You did this back in 2011 when Pharmac staff reported to the Board that there would be up to 100 affected patients in New Zealand with Pompe disease. That treatment was declined, and it looks as though you intend to decline Soliris, again with dubious data as the basis for a decision. This is not acceptable and you should withdraw this consultation because of the misleading information in it, which is likely to skew responses from the public. .

- These patients have a right to life. Our health system has a duty to address their needs in a fair and equitable way. When Pharmac places so much emphasis on costs, cost-effectiveness and alternative use of the money, but does not address issues of rights, equity, fairness and community values, you are failing in your duty. The District Health Boards have this duty, and Pharmac is acting as their purchasing agency. You should use the same decision criteria and priorities that they have.

- Patients with rare diseases are disadvantaged because their condition is rare, and when treatments become available they are doubly affected by the high cost and the very small market due to small numbers. It is a denial of their right to health, and contrary to goals of "Equity of access, reducing inequalities and improving health outcomes for individuals and communities will guide our relationship and decision making", which are set out in the agreement between DHBs and Pharmac about how you decide things on their behalf.

- **Decision Criteria:** The decision criteria used to assess medicines are not fair for those who are affected by rare diseases. There should be an additional layer of decision-making for rare diseases that do not fit the standard cost effectiveness threshold for large populations. These additional layers already exist in Australia, England, Scotland and other places around the world, because they have recognised that it's a fair way to deal with the disadvantage of rare diseases.

- Pharmac's assumption that "best health outcomes" as mentioned in its legislation, can be strongly associated with calculation of Quality Adjusted Life Years, is too narrow a view of what is "best", especially when Pharmac's calculations do not take into account non-health-sector costs. The decision criteria should include a broader range of considerations that are important to patients, so that the decisions are made in a patient-centred way.

- Pharmac also has a responsibility for "health outcomes that are reasonably achievable". Your narrow perspective on technical assessment and budget management, to the exclusion of patient rights and interests from decisions, are an outcome that effectively discriminates against patients with rare diseases, and is not a reasonable outcome by any measure.

- Pharmac often emphasises the "tough decision" approach that some will be funded and others not. That is too simplistic and can also be unfair, as it is in this case. There are very good reasons to adopt an equitable approach that spreads medicine funding across as many areas of health need as possible so that no group is completely abandoned. That is the approach across a wide range of services in our health system and Pharmac should adopt a similar approach, rather than exclude and abandon certain groups.

- __**I do not support**__ your intent to decline treatment for this group or other patients where there are therapies for rare diseases.

Gil Hardy PhD FRSC

Professor of Clinical Nutrition

Te Kura Hangarau o Kai-oranga-a-tangata

Massey University Albany Campus

[redacted]

[redacted]

From: [redacted]
Sent: Wednesday, 5 June 2013 9:44 a.m.
To: eculizumabfeedback
Subject: Submission to PHARMAC's Proposal to decline a funding application for Soliris (eculizumab)

To Whom it May Concern;

I support PNH patients in New Zealand in their fight to gain access to the life saving treatment Soliris.

I DO NOT support PHARMAC's proposal to decline funding for the Soliris treatment.

I support the PNH Support Association's proposition for Equity and Fairness which states:

i. **PHARMAC must return to the negotiating table with the supplier of the Soliris treatment**

ii. **PHARMAC must negotiate in good faith toward funding Soliris for a minimum of 8 NZ PNH patients**

iii. **PHARMAC must establish fair assessment criteria, based on expert advice from the international haematological community, to assess patient need for the Soliris treatment**

iv. **PHARMAC must amend its Operating Policies and Procedures to acknowledge the right of rare disease patients to access life restoring and life saving treatments as in the specific example of the Soliris treatment**

One of these men is the Father of a little boy I know. If you don't care about the lives of the adults at least think of the lives of their children and the family members they will leave behind. Especially when this CAN be prevented!

Yours faithfully

[redacted]

From: [redacted]
Sent: Tuesday, 30 July 2013 1:00 p.m.
To: eculizumabfeedback
Subject: Submission to PHARMAC's Proposal to decline a funding application for Soliris (eculizumab)
Attachments: proposition_equity_and_fairness_-_email_submission_template.doc

To:

Sue Anne Yee
Therapeutic Group Manager
PHARMAC

29th July 2013

To Whom it May Concern;

I support PNH patients in New Zealand in their fight to gain access to the life saving treatment Soliris.

I DO NOT support PHARMAC's proposal to decline funding for the Soliris treatment.

I support the PNH Support Association's proposition for Equity and Fairness which states:

i. **PHARMAC must return to the negotiating table** with the supplier of the Soliris treatment

ii. **PHARMAC must negotiate in good faith** toward funding Soliris for a minimum of 8 NZ PNH patients

iii. **PHARMAC must establish fair assessment criteria**, based on expert advice from the international haematological community, to assess patient need for the Soliris treatment

iv. **PHARMAC must amend its Operating Policies and Procedures** to acknowledge the right of rare disease patients to access life restoring and life saving treatments as in the specific example of the Soliris treatment

As parents with a [redacted]*, we know first hand what a difference to our daughters life, receiving vital treatment makes. She currently receives* [redacted]*, without which she would most likely not be alive today. However, with the* [redacted] *she has managed to live a healthy and almost normal life with all the opportunities this provides. We are aware of the costs of this and we appreciate the help she receives, but also the difficult position PHARMAC is in. With this in mind we are very sad to hear that PHARMAC is considering declining it's funding for Soliris and thereby condeming patients to a life of suffereing and possibly death. Whilst it is unfair that the drug companies charge so much for some of these medicines, it is also unfair that PHARMAC decides who lives and who dies just because of budget constraints. If the money is not there, then the government should and must provide more, regardless of who they are and what their condition is. That is the role of a socially responsible government.*

We seriously hope you reconsider this decision.

Yours faithfully,

[redacted]

From: Greg Taylor [redacted]
Sent: Wednesday, 31 July 2013 3:08 p.m.
To: eculizumabfeedback

Greedy fucks, all you care about is money and yourself.

From: Tony Pearson [redacted]
Sent: Tuesday, 30 July 2013 8:55 p.m.
To: eculizumabfeedback
Subject: Attn Ms Yee
Attachments: Letter to Pharmac 30.7.13.doc

Dear Ms Yee
Please find attached a submission from the Guillain Barre Syndrome Support Group NZ in respect of your intention to decline funding for Soliris
A.R.Pearson
Secretary

GBS New Zealand

Guillain–Barré Syndrome
Support Group New Zealand Trust
www.gbsnz.org.nz

Registered N.Z. Charity No. CC20639 Charities Act 2005

Sue Anne Yee
Therapeutic Group Manager
PHARMAC

PO Box 10 254, Wellington 6143

30th July 2013.

Subject: **<u>Submission to PHARAMC'S proposal to decline a funding application for Soliris (eculizumab)</u>**

Dear Ms Yee

We support the PNH patients in New Zealand in their fight to gain access to the lifesaving treatment Soliris.

We **DO NOT** support Pharmac's proposed intent to decline this treatment

Patients with rare diseases are disadvantaged because their condition is rare, and when treatments become available they are doubly affected by the high cost and the very small market due to small numbers. It is a denial of their right to health, and contrary to goals of "Equity of access, reducing inequalities and improving health outcomes for individuals and communities will guide our relationship and decision making", which are set out in the agreement between DHBs and Pharmac about how you decide things on their behalf.

The decision criteria used to assess medicines are not fair for those who are affected by rare diseases. There should be an additional layer of decision-making for rare diseases that do not fit the standard cost effectiveness threshold for large populations. These additional layers already exist in Australia, England, Scotland and other places around the world, because they have recognised that it's a fair way to deal with the disadvantage of rare diseases.

Pharmac also has a responsibility for "health outcomes that are reasonably achievable". Your narrow perspective on technical assessment and budget management, to the exclusion of patient rights and interests from decisions, are an outcome that effectively discriminates against patients with rare diseases, and is not a reasonable outcome by any measure.

Pharmac often emphasises the "tough decision" approach that some will be funded and others not. That is too simplistic and can also be unfair, as it is in this case. There are very good reasons to adopt an equitable approach that spreads medicine funding across as many areas of health need as possible so that no group is completely abandoned. That is the approach across a wide range of services in our health system and Pharmac should adopt a similar approach, rather than exclude and abandon certain groups.

<u>We do not support</u> your intent to decline treatment for this group or other patients where there are therapies for rare diseases.

Anthony R Pearson
Secretary

From: Hamish Williamson [redacted]
Sent: Saturday, 20 July 2013 3:51 p.m.
To: eculizumabfeedback; OPP Review
Subject: Soliris

Re: Submission opposing PHARMAC's Proposal to decline a funding application for Soliris (eculizumab)

To Whom it May Concern;

I SUPPORT PNH patients in New Zealand in their fight to gain access to the lifesaving treatment Soliris.

I OPPOSE PHARMAC's proposal to decline funding for the Soliris treatment.

I support the PNH Support Association's proposition for Equity and Fairness which states:

1. **PHARMAC must return to the negotiating table** with the supplier of the Soliris treatment
2. **PHARMAC must negotiate in good faith** toward funding Soliris for a minimum of 8 NZ PNH patients
3. **PHARMAC must establish fair assessment criteria**, based on expert advice from the international haematological community, to assess patient need for the Soliris treatment
4. **PHARMAC must amend its Operating Policies and Procedures** to acknowledge the right of rare disease patients to access life restoring and lifesaving treatments as in the specific example of the Soliris treatment

Please do the human thing and consider the plight of the unfortunate people suffering from PNH, and do what you can to help.
Thanks.

Yours faithfully

Hamish Williamson.

From:	Heather Morgan [redacted]
Sent:	Tuesday, 30 July 2013 9:28 p.m.
To:	eculizumabfeedback
Subject:	Submission to PHARMAC's Proposal to decline a funding application for Soliris (eculizumab)

To Whom it May Concern;

I support PNH patients in New Zealand in their fight to gain access to the life saving treatment Soliris.

Whilst I understand there are many factors to consider, I would respectfully beseech you to return to the negotiating table with the supplier of the Soliris treatment and negotiate toward funding Soliris for those patients needing this to survive.

I support the PNH Support Association's proposition for Equity and Fairness which states:

i. **PHARMAC must return to the negotiating table** with the supplier of the Soliris treatment

ii. **PHARMAC must negotiate in good faith** toward funding Soliris for a minimum of 8 NZ PNH patients

iii. **PHARMAC must establish fair assessment criteria,** based on expert advice from the international haematological community, to assess patient need for the Soliris treatment

iv. **PHARMAC must amend its Operating Policies and Procedures** to acknowledge the right of rare disease patients to access life restoring and life saving treatments as in the specific example of the Soliris treatment

These patients deserve a chance to live – they can not negotiate with the supplier themselves and must rely on PHARMAC to do it for them. Whilst it may be unreasonable to fund this drug for such a small number over benefits for 40,000 others, this will still mean that somebody's child, brother, sister, parent will die as a result. Please continue to negotiate until it is affordable to fund.

Yours faithfully

H Morgan

From: [redacted]
Sent: Sunday, 2 June 2013 2:41 p.m.
To: eculizumabfeedback
Subject: Soliris

To Whom it May Concern;

I support PNH patients in New Zealand in their fight to gain access to the life saving treatment Soliris.

I DO NOT support PHARMAC's proposal to decline funding for the Soliris treatment.

I support the PNH Support Association's proposition for Equity and Fairness which states:

i. **PHARMAC must return to the negotiating table with the supplier of the Soliris treatment**

ii. **PHARMAC must negotiate in good faith toward funding Soliris for a minimum of 8 NZ PNH patients**

iii. **PHARMAC must establish fair assessment criteria, based on expert advice from the international haematological community, to assess patient need for the Soliris treatment**

iv. **PHARMAC must amend its Operating Policies and Procedures to acknowledge the right of rare disease patients to access life restoring and life saving treatments as in the specific example of the Soliris treatment**

This drug is life-saving. It is not a perk, or something that it perhaps might be nice to have; it is necessary to save these people's lives. What price someone's life? If it were your child, and he was ill with this disease, and there was treatment available... but you unfortunately weren't a millionaire... would you throw up your hands, say "I can't afford it", and let him die? I know the parents of [redacted], one of these eight people. They are nice people. They are loving people. They are the sort of people who will give anything they can... but they are not rich. And they are stressed out of their brains trying to save the life of their wonderful son so that their grandchild can keep his father. What price [redacted]'s life? What price the lives of the others who suffer? Find your humanity. Have compassion on these people who are going to die... if you do nothing. You have the potential to save lives, real human lives. Why would you walk away?

Yours faithfully

[redacted]

From: Heidi Brickell [redacted]
Sent: Tuesday, 30 July 2013 1:11 p.m.
To: eculizumabfeedback
Subject: Letter of support for the funding of Soliris in New Zealand for PNH Sufferers

To Whom it May Concern;

I support PNH patients in New Zealand in their fight to gain access to the life saving treatment Soliris.

I DO NOT support PHARMAC's proposal to decline funding for the Soliris treatment.

I support the PNH Support Association's proposition for Equity and Fairness which states:

i. **PHARMAC must return to the negotiating table** with the supplier of the Soliris treatment

ii. **PHARMAC must negotiate in good faith** toward funding Soliris for a minimum of 8 NZ PNH patients

iii. **PHARMAC must establish fair assessment criteria**, based on expert advice from the international haematological community, to assess patient need for the Soliris treatment

iv. **PHARMAC must amend its Operating Policies and Procedures** to acknowledge the right of rare disease patients to access life restoring and life saving treatments as in the specific example of the Soliris treatment

It is not acceptable to risk these people's lives by jeapardising their access to treatment. I have also heard that the actual price that has been offered to you by the company Alexion is significantly lower than what you, Pharmac, have been publicizing. I know the real cost that has been offered by the company can't be published because of confidentiality, but it is so unfair of you to misrepresent this information in a consultuation to the public.

You are a department hired by the government, whose responsibility it is to provide accessible health care, and quality of life to those who need it. There are enough funds to treat people for this disease which is not their fault. Why not borrow from some of the drugs aimed more at the baby boomers, who own two homes already, and spend their lives on cruise ships?

Soliris is funded in almost every Western country as well as in Asia and the Middle east, I urge you to look at the human issue, in these economic consideration,s and the social justice in considering how unfair it is to deny thse people access based on the unfortunate rarity of the diseas, which they can not help.

Yours faithfully

Heidi Brickell

From: Helana Middlemiss [redacted]
Sent: Tuesday, 16 July 2013 7:55 a.m.
To: eculizumabfeedback; OPP Review
Subject: Submission opposing PHARMAC's Proposal to decline a funding application for Soliris (eculizumab)

Submission opposing PHARMAC's Proposal to decline a funding application for Soliris (eculizumab)

To Whom it May Concern;

I SUPPORT PNH patients in New Zealand in their fight to gain access to the lifesaving treatment Soliris.

I OPPOSE PHARMAC's proposal to decline funding for the Soliris treatment.

I support the PNH Support Association's proposition for Equity and Fairness which states:

i. **PHARMAC must return to the negotiating table** with the supplier of the Soliris treatment

ii. **PHARMAC must negotiate in good faith** toward funding Soliris for a minimum of 8 NZ PNH patients

iii. **PHARMAC must establish fair assessment criteria,** based on expert advice from the international haematological community, to assess patient need for the Soliris treatment

iv. **PHARMAC must amend its Operating Policies and Procedures** to acknowledge the right of rare disease patients to access life restoring and lifesaving treatments as in the specific example of the Soliris treatment

Yours faithfully

Helana Middlemiss

[redacted]

[redacted]

From: Helena Skalova [redacted]
Sent: Saturday, 1 June 2013 9:15 p.m.
To: eculizumabfeedback
Subject: submission

To Whom it May Concern;

I support PNH patients in New Zealand in their fight to gain access to the life saving treatment Soliris.

I DO NOT support PHARMAC's proposal to decline funding for the Soliris treatment.

I support the PNH Support Association's proposition for Equity and Fairness which states:

i. PHARMAC must return to the negotiating table with the supplier of the Soliris treatment

ii. PHARMAC must negotiate in good faith toward funding Soliris for a minimum of 8 NZ PNH patients

iii. PHARMAC must establish fair assessment criteria, based on expert advice from the international haematological community, to assess patient need for the Soliris treatment

iv. PHARMAC must amend its Operating Policies and Procedures to acknowledge the right of rare disease patients to access life restoring and life saving treatments as in the specific example of the Soliris treatment

Yours sincerely

Helena Skalova

From: [redacted] [redacted]
Sent: Monday, 8 July 2013 11:36 a.m.
To: eculizumabfeedback
Subject: Submission to PHARMAC's Proposal to decline a funding application for Soliris (eculizumab)

To whom it concerns,

Regarding PHARMAC's intention not to fund Soliris:

I believe the above stated intention is wrong for the following reasons:

First and foremost the Soliris treatment is a life saving treatment. *It works!* It extends the life of PNH patients by an average of 32.5 years.
These patients have the highest need for this treatment. Unlike other disease's there are no other treatment options for PNH suffers, therefore it is the duty of the New Zealand Government (by extension PHARMAC) to provide the Soliris treatment to those New Zealanders that need it.

PHARMAC raised the possible "health gains" of a non-specified group of patients in a non-specified area with relation to this funding level in it's, call for submissions.
To be clear we are not talking about "health gains" for New Zealand's PNH sufferers. We are talking about living or dying! For this reason above all else the Soliris treatment must be funded!

In the call for submissions on this issue, PHARMAC expressed concern about giving PNH patients and their families "certainty about a decision". I am certain, that PNH patients and their families would far prefer the certainty that they will be alive rather than that a decision has been made.

The fact that PNH is a rare condition and affects a small number of New Zealanders should most definitely *not* be a factor in the decision-making criteria. What is important is that the Soliris treatment is the solution for PNH sufferers, regardless if they are few or many. The Soliris treatment restores normal life expectancy and allows normal functioning for PNH suffers. It works, it's proven, it must be provided!

There is no alternative for those afflicted by PNH, there are no other options to consider or evaluate. It comes down to having Soliris treatment and living, or not having Soliris treatment and dying 30+ years to soon.

The above facts, which are endorsed by every expert in the PHN field the world over, must be the basis of the decision weather to fund the treatment. New Zealand has the ability to save the lives of these citizens and that must be the overridingfactor in this process. The Soliris treatment must be funded!

The major reason given by PHARMAC for denying New Zealand's PNH suffers treatment is cost. Cost must *not* be a factor in deciding whether to provide New Zealand citizens a proven, life saving treatment that they deserve. The issue of cost should be handled *after* the right to receive this life saving treatment is delivered to these citizens by their government.

In this situation it is up to PHARMAC to negotiate, on behalf of New Zealanders, the most advantageous deal to supply the treatment, and not to deny New Zealanders life because they could not negotiate a solution.

PHARMAC must return to negotiations with the mandate to find a solution.

PHARMAC must establish fair assessment criteria, based on expert advice from the international haematological community, to assess patient need for the Soliris treatment

PHARMAC must amend its Operating Policies and Procedures to acknowledge the right of rare disease patients to access life restoring and life saving treatments as in the specific example of the Soliris treatment

Sincerely and with the utmost conviction,

[redacted]

[redacted]

From: J Kerr [redacted]
Sent: Tuesday, 4 June 2013 8:29 p.m.
To: eculizumabfeedback
Subject: Submission to PHARMAC's Proposal to decline a funding

To Whom it May Concern;

I support PNH patients in New Zealand in their fight to gain access to the life saving treatment Soliris.

I DO NOT support PHARMAC's proposal to decline funding for the Soliris treatment.

I support the PNH Support Association's proposition for Equity and Fairness which states:

i. **PHARMAC must return to the negotiating table** with the supplier of the Soliris treatment

ii. **PHARMAC must negotiate in good faith** toward funding Soliris for a minimum of 8 NZ PNH patients

iii. **PHARMAC must establish fair assessment criteria**, based on expert advice from the international haematological community, to assess patient need for the Soliris treatment

iv. **PHARMAC must amend its Operating Policies and Procedures** to acknowledge the right of rare disease patients to access life restoring and life saving treatments as in the specific example of the Soliris treatment

Save lives, do the right thing!

Yours faithfully

JK.

From: Jade Farley [redacted]
Sent: Monday, 17 June 2013 11:47 a.m.
To: eculizumabfeedback
Subject: Submission to PHARMAC's Proposal to decline a funding application for Soliris (eculizumab)

To Whom it May Concern;

I support PNH patients in New Zealand in their fight to gain access to the life saving treatment Soliris.

I DO NOT support PHARMAC's proposal to decline funding for the Soliris treatment.

I support the PNH Support Association's proposition for Equity and Fairness which states:

i. **PHARMAC must return to the negotiating table** with the supplier of the Soliris treatment

ii. **PHARMAC must negotiate in good faith** toward funding Soliris for a minimum of 8 NZ PNH patients

iii. **PHARMAC must establish fair assessment criteria**, based on expert advice from the international haematological community, to assess patient need for the Soliris treatment

iv. **PHARMAC must amend its Operating Policies and Procedures** to acknowledge the right of rare disease patients to access life restoring and life saving treatments as in the specific example of the Soliris treatment

This issue directly affect somebody I know personally and I urge PHARMAC to urgently reconsider it's funding decision as I believe everyone should have the right to life saving medicine in New Zealand.

Yours faithfully

Jade Farley

From: James Frovich [redacted]
Sent: Tuesday, 4 June 2013 4:43 p.m.
To: eculizumabfeedback
Subject: This is unacceptable to let people die when a life saving treatment is available.
Attachments: proposition_equity_and_fairness_-_email_submission_template.doc

This is unacceptable to let people die when a life saving treatment is available. Let them live!

Please email *BOTH*

Subject: Submission to PHARMAC's Proposal to decline a funding application for Soliris (eculizumab)
Email: eculizumabfeedback@pharmac.govt.nz

AND

Subject: Submission in response to PHARMAC's consultation on Decision Critera.
Email: opp@pharmac.govt.nz

To Whom it May Concern;

I support PNH patients in New Zealand in their fight to gain access to the life saving treatment Soliris.

I DO NOT support PHARMAC's proposal to decline funding for the Soliris treatment.

I support the PNH Support Association's proposition for Equity and Fairness which states:

i. **PHARMAC must return to the negotiating table** with the supplier of the Soliris treatment

ii. **PHARMAC must negotiate in good faith** toward funding Soliris for a minimum of 8 NZ PNH patients

iii. **PHARMAC must establish fair assessment criteria**, based on expert advice from the international haematological community, to assess patient need for the Soliris treatment

iv. **PHARMAC must amend its Operating Policies and Procedures** to acknowledge the right of rare disease patients to access life restoring and life saving treatments as in the specific example of the Soliris treatment

(please include your personal statements here)

Yours faithfully

Please email *BOTH*

Subject: Submission to PHARMAC's Proposal to decline a funding application for Soliris (eculizumab)
Email: eculizumabfeedback@pharmac.govt.nz

AND

Subject: Submission in response to PHARMAC's consultation on Decision Critera.
Email: opp@pharmac.govt.nz

To Whom it May Concern;

I support PNH patients in New Zealand in their fight to gain access to the life saving treatment Soliris.

I DO NOT support PHARMAC's proposal to decline funding for the Soliris treatment.

I support the PNH Support Association's proposition for Equity and Fairness which states:

i. **PHARMAC must return to the negotiating table** with the supplier of the Soliris treatment

ii. **PHARMAC must negotiate in good faith** toward funding Soliris for a minimum of 8 NZ PNH patients

iii. **PHARMAC must establish fair assessment criteria**, based on expert advice from the international haematological community, to assess patient need for the Soliris treatment

iv. **PHARMAC must amend its Operating Policies and Procedures** to acknowledge the right of rare disease patients to access life restoring and life saving treatments as in the specific example of the Soliris treatment

(please include your personal statements here)

Yours faithfully

From: [redacted] [redacted]
Sent: Thursday, 18 July 2013 10:42 p.m.
To: eculizumabfeedback
Cc: [redacted]
Subject: Submission to PHARMAC's Proposal to decline a funding application for Soliris (eculizumab)

To whom it may concern.

I am writing this submission to you as the New Zealand born partner of a current sufferer from the PNH condition.
[redacted] was diagnosed with this condition some 15years ago and has lived and dealt with the inherent issues in the intervening years. It has now come to a point where there is little hope of any remission and [redacted] she has been forced to consider the life-changing medication Soliris. I use the term "life-changing" not only to highlight the effect this medication will have on her own personal health and well-being but also because she will effectively be exiled from her home country and her family because of your current stance on the funding of this medication.

[redacted] and I both live in [redacted]. Due to the personal commitment I have to [redacted] I too will effectively be exiled from my homeland and my family as well. I have [redacted] all with families of their own.

[redacted] finds herself in a very lucky position to be eligible to benefit from this medication; my thoughts go out to her fellow sufferers in New Zealand who currently do not have this opportunity. They face a very uncertain future and this must place a huge strain on not only their own health and piece of mind but also that of their families, dependants and friends. No-one should be put in a position where their very existence is controlled by a state-run agency whereas in comparison other first world countries around the world provide an appropriate level of care for those unfortunate enough to suffer from PNH and other rare conditions.

Whilst I understand that the cost of this medication is expensive I consider that you are effectively putting a price on someone's quality of life and in some cases, on their life itself.

I support PNH patients in New Zealand in their fight to gain access to the life-saving treatment Soliris.
I DO **NOT** support PHARMAC's proposal to decline funding for the Soliris treatment.
I support the PNH Support Association's proposition for Equity and Fairness which states:

i. PHARMAC must return to the negotiating table with the supplier of the Soliris treatment.

ii. PHARMAC must negotiate in good faith toward funding Soliris for a minimum of 8 NZ PNH patients
iii. PHARMAC must establish fair assessment criteria, based on expert advice from the international haematological community, to assess patient need for the Soliris treatment
iv. PHARMAC must amend its Operating Policies and Procedures to acknowledge the right of rare disease patients to access life restoring and life-saving treatments as in this specific example of the Soliris treatment

Yours sincerely,

[redacted]

[redacted]

From:	Jan Marner [redacted]
Sent:	Wednesday, 5 June 2013 2:14 p.m.
To:	eculizumabfeedback
Subject:	submission
Attachments:	proposition_equity_and_fairness_-_email_submission_template.doc

Please email *BOTH*

Subject: Submission to PHARMAC's Proposal to decline a funding application for Soliris (eculizumab)
Email: eculizumabfeedback@pharmac.govt.nz

AND

Subject: Submission in response to PHARMAC's consultation on Decision Critera.
Email: opp@pharmac.govt.nz

To Whom it May Concern;

I support PNH patients in New Zealand in their fight to gain access to the life saving treatment Soliris.

I DO NOT support PHARMAC's proposal to decline funding for the Soliris treatment.

I support the PNH Support Association's proposition for Equity and Fairness which states:

i. **PHARMAC must return to the negotiating table** with the supplier of the Soliris treatment

ii. **PHARMAC must negotiate in good faith** toward funding Soliris for a minimum of 8 NZ PNH patients

iii. **PHARMAC must establish fair assessment criteria**, based on expert advice from the international haematological community, to assess patient need for the Soliris treatment

iv. **PHARMAC must amend its Operating Policies and Procedures** to acknowledge the right of rare disease patients to access life restoring and life saving treatments as in the specific example of the Soliris treatment

It is sick & unacceptable that this has not been funded already. These poor people, knowing that there is help for them, but our government does not care enough about them. Maybe if we stopped sending so much money overseas every year we would have more to look after our own!!! I only came to know about this due to a news article about a little boy who needs this medicine. I DO NOT know him personally, but even I care enough to make weekly donations to his cause, I have spread the word about it & have returned this submission. I am but one person doing what I can! PHARMAC should and COULD be doing a lot more!!!

Shame on PHARMAC!!!

Yours faithfully

Jan Marner.

From: Jane Heyward [redacted]
Sent: Friday, 21 June 2013 2:46 a.m.
To: OPP Review; eculizumabfeedback
Subject: Submission to PHARMAC's Proposal to decline a funding application for Soliris (eculizumab)

To Whom it May Concern;

I support PNH patients in New Zealand in their fight to gain access to the life saving treatment Soliris.

I DO NOT support PHARMAC's proposal to decline funding for the Soliris treatment.

I support the PNH Support Association's proposition for Equity and Fairness which states:

i. PHARMAC must return to the negotiating table with the supplier of the Soliris treatment

ii. PHARMAC must negotiate in good faith toward funding Soliris for a minimum of 8 NZ PNH patients

iii. PHARMAC must establish fair assessment criteria, based on expert advice from the international haematological community, to assess patient need for the Soliris treatment

iv. PHARMAC must amend its Operating Policies and Procedures to acknowledge the right of rare disease patients to access life restoring and life saving treatments as in the specific example of the Soliris treatment

Why on earth would you deny treatment?

Yours faithfully

Jane Heyward
[redacted]

[redacted]

[redacted]

From: Rob Lawson [redacted]
Sent: Friday, 19 July 2013 1:57 p.m.
To: eculizumabfeedback
Attachments: Pharmac's intent to decline Soliris 2013.docx

Jane Lawson
[redacted]

To provide feedback, please submit it in writing by **Wednesday, 31 July 2013** to:

Sue Anne Yee
Therapeutic Group Manager
PHARMAC

Email: eculizumabfeedback@pharmac.govt.nz
Fax: 04 460 4995
Post: PO Box 10 254, Wellington 6143

Subject: **Submission to PHARAMC'S proposal to decline a funding application for Soliris (eculizumab)**

To whom it may concern:

I support the PNH patients in New Zealand in their fight to gain access to the lifesaving treatment Soliris.

I **DO NOT** support Pharmac's proposed intent to decline this treatment.

- When Pharamc takes this type of consultation to the public I should be able to rely on Pharmac to provide accurate and reliable information that will help guide responses from the community. **In this consultation you have overstated the number of people affected with PNH which in turn over exaggerates the likely real cost of treatment.** You did this back in 2011 when Pharmac staff reported to the Board that there would be up to 100 affected patients in New Zealand with Pompe disease. That treatment was declined, and it looks as though you intend to decline Soliris, again with dubious data as the basis for a decision. This is not acceptable and withdraw this consultation as the misleading information in it, is likely to skew responses from the public.

- Patients with rare diseases are denied of their right to health, and contrary to goals of "Equity of access, reducing inequalities and improving health outcomes for individuals and communities will guide our relationship and decision making", which are set out in the agreement between DHBs and Pharmac about how you decide things on their behalf.

- **Decision Criteria:** The decision criteria used to assess medicines are not fair for those who are affected by rare diseases in New Zealand. There should be an additional layer of decision-making for rare diseases that do not fit the standard cost effectiveness threshold for large populations. These additional layers already exist in Australia, England, Scotland and other places around the world, because they have recognised that it's a fair way to deal with the disadvantage of rare diseases.

I do not support your intent to decline treatment for the PNH group or other patients where there are therapies for rare diseases.

From: [redacted] [redacted]
Sent: Wednesday, 31 July 2013 5:27 p.m.
To: eculizumabfeedback
Subject: Proposal to decline funding for Soliris
Attachments: PHARMAC soliris submission.docx

Please see attached my submission against the current proposal.

[redacted]

Sent from my iPhone

31 July 2013

To Whom It May Concern

I am writing in support of New Zealand PNH sufferers seeking funding of the drug Soliris following news of PHARMAC's proposal to reject funding for it. I do so as a result of my first hand experience watching the deterioration of the health of a very good friend who has this condition, [redacted].

[redacted]

I now know, following her diagnosis some years ago, that these are just two of her symptoms of PNH and that they are far more serious than I could have guessed at the time – [redacted] now requires fairly frequent blood transfusions when her condition deteriorates, usually as a result of an infection that most of us would easily shrug off. She has also been advised by her medical care that she is at increased risk of other forms of blood clots which are a considerable danger to her. These symptoms, [redacted] have become far more commonplace, prompting her doctors to urge her onto Soliris.

This treatment prevents the destruction of red blood cells, meaning that patients have less fatigue and require blood transfusions either less or not at all. As she currently lives in [redacted] the treatment is funded by [redacted]. However, the sheer cost of Soliris without funding is completely prohibitive. In addition, the treatment must be continued for life as once commenced there is the risk of increased destruction of red blood cells, worsening the condition.

While any treatment [redacted] receives is currently under the [redacted], if PHARMAC does not extend funding to Soliris, she will be unable to return to NZ to live and work (I understand that New Zealand is the only OCHD country not to apply funding for this drug). This in effect places her "in exile" to use her own words. I too am a New Zealander living abroad [redacted]. While currently family and work keep me here, I would hope one day to be able to return to work in New Zealand. If I had a condition such as PNH that required certain treatment, I would hope that I would have access to that treatment in my home country, and not feel that I had to live away from family and my support system. This is what I hope for [redacted], who has gone through so much on her own over in [redacted]. Without it, she faces, amongst other things, reduced lifespan, and reduced quality of life during the time she has.

Given the extensive funding made for drugs for lifestyle conditions, I do not find this a fair or just approach – those with PNH do not contribute or cause their condition through diet and lifestyle. In fact [redacted] is and always has been a healthy active person until PNH effects prohibited it.

[redacted] is a smart, intelligent, motivated person who would love to return to NZ in the future. The skills she has acquired both in her professional career [redacted] and as a participant in the PNH support system in [redacted] are highly valuable to New Zealand and would be wasted if she were never able to return to pass those on due to her health requirements.

I understand that PHARMAC has itself recognised the benefits Soliris can have to PNH sufferers. I request that you do not discriminate against New Zealanders who are unfortunate enough to have a rare disease and that you reconsider your proposal not to fund this treatment.

I support PNH patients in New Zealand in their fight to gain access to the life saving treatment Soliris.

I DO NOT support PHARMAC's proposal to decline funding for the Soliris treatment.

I support the PNH Support Association's proposition for Equity and Fairness which states:

i. **PHARMAC must return to the negotiating table** with the supplier of the Soliris treatment

ii. **PHARMAC must negotiate in good faith** toward funding Soliris for a minimum of 8 NZ PNH patients

iii. **PHARMAC must establish fair assessment criteria**, based on expert advice from the international haematological community, to assess patient need for the Soliris treatment

iv. **PHARMAC must amend its Operating Policies and Procedures** to acknowledge the right of rare disease patients to access life restoring and life saving treatments as in the specific example of the Soliris treatment

Yours faithfully

[redacted]

From: Janine Clarkson [redacted]
Sent: Tuesday, 4 June 2013 8:06 p.m.
To: eculizumabfeedback
Subject: Submission to PHARMAC's Proposal to decline a funding application for Soliris (eculizumab)

--

To Whom it May Concern;

I support PNH patients in New Zealand in their fight to gain access to the life saving treatment Soliris.

I DO NOT support PHARMAC's proposal to decline funding for the Soliris treatment.

I support the PNH Support Association's proposition for Equity and Fairness which states:

1. **PHARMAC must return to the negotiating table with the supplier of the Soliris treatment**
2. **PHARMAC must negotiate in good faith toward funding Soliris for a minimum of 8 NZ PNH patients**
3. **PHARMAC must establish fair assessment criteria, based on expert advice from the international haematological community, to assess patient need for the Soliris treatment**
4. **PHARMAC must amend its Operating Policies and Procedures to acknowledge the right of rare disease patients to access life restoring and life saving treatments as in the specific example of the Soliris treatment**

It only has to save one child and it is worth it.

Yours faithfully

Janine Clarkson

--
Janine Clarkson
[redacted]

From: [redacted]
Sent: Monday, 22 July 2013 8:48 p.m.
To: eculizumabfeedback
Subject: Voicing our displeasure
Attachments: Pharmac's intent to decline Soliris 2013.docx

To whom it may concern;
Find attached a formal submission which outlines not only my feeling and opinions but my families and friends. We have a son who recently has been diagnosed with a rare disease and understand first hand the trials and pressure this puts people under. To take away any hope families have for the week being of there children (no matter what the cost) is wrong and not what this society/country is about.
I thank you for your time, and I encourage you to think long and hard about what precedent may be set, and how you would feel if you were in our position.
Thanks again and if you have any questions feel free to contact me.
Cheers
[redacted]
Sent from my Motorola ATRIX2

To whom it may concern:

I support the PNH patients in New Zealand in their fight to gain access to the lifesaving treatment Soliris.

I **DO NOT** support Pharmac's proposed intent to decline this treatment.

- When Pharamc takes this type of consultation to the public I should be able to rely on Pharmac to provide accurate and reliable information that will help guide responses from the community. In this consultation you have overstated the number of people affected with PNH which in turn over exaggerates the likely real cost of treatment. You did this back in 2011 when Pharmac staff reported to the Board that there would be up to 100 affected patients in New Zealand with Pompe disease. That treatment was declined, and it looks as though you intend to decline Soliris, again with dubious data as the basis for a decision. This is not acceptable and you should withdraw this consultation because of the misleading information in it, which is likely to skew responses from the public.

- These patients have a right to life. Our health system has a duty to address their needs in a fair and equitable way. When Pharmac places so much emphasis on costs, cost-effectiveness and alternative use of the money, but does not address issues of rights, equity, fairness and community values; you are failing in your duty. The District Health Boards have this duty, and Pharmac is acting as their purchasing agency. You should use the same decision criteria and priorities that they have.

- Patients with rare diseases are disadvantaged because their condition is rare, and when treatments become available they are doubly affected by the high cost and the very small market due to small numbers. It is a denial of their right to health, and contrary to goals of "Equity of access, reducing inequalities and improving health outcomes for individuals and communities will guide our relationship and decision making", which are set out in the agreement between DHBs and Pharmac about how you decide things on their behalf.

- **Decision Criteria:** The decision criteria used to assess medicines are not fair for those who are affected by rare diseases. There should be an additional layer of decision-making for rare diseases that do not fit the standard cost effectiveness threshold for large populations. These additional layers already exist in Australia, England, Scotland and other places around the world, because they have recognised that it's a fair way to deal with the disadvantage of rare diseases.

- Pharmac's assumption that "best health outcomes" as mentioned in its legislation, can be strongly associated with calculation of Quality Adjusted Life Years, is too narrow a view of what is "best", especially when Pharmac's calculations do not take into account non-health-sector costs. The decision criteria should include a broader range of considerations that are important to patients, so that the decisions are made in a patient-centred way.
- Pharmac also has a responsibility for "health outcomes that are reasonably achievable". Your narrow perspective on technical assessment and budget management, to the exclusion of patient rights and interests from decisions, are an outcome that effectively discriminates against patients with rare diseases, and is not a reasonable outcome by any measure.
- Pharmac often emphasises the "tough decision" approach that some will be funded and others not. That is too simplistic and can also be unfair, as it is in this case. There are very good reasons to adopt an equitable approach that spreads medicine funding across as many areas of health need as possible, so that no group is completely abandoned. That is the approach across a wide range of services in our health system and Pharmac should adopt a similar approach, rather than exclude and abandon certain groups.

I do not support your intent to decline treatment for the PNH group or other patients where there are therapies for rare diseases.

From: Jen Schroder [redacted]
Sent: Monday, 3 June 2013 5:53 p.m.
To: eculizumabfeedback; OPP Review
Subject: Soliris

To Whom it May Concern;

I support PNH patients in New Zealand in their fight to gain access to the life saving treatment Soliris.

I DO NOT support PHARMAC's proposal to decline funding for the Soliris treatment.

I support the PNH Support Association's proposition for Equity and Fairness which states:

i. **PHARMAC must return to the negotiating table** with the supplier of the Soliris treatment

ii. **PHARMAC must negotiate in good faith** toward funding Soliris for a minimum of 8 NZ PNH patients

iii. **PHARMAC must establish fair assessment criteria**, based on expert advice from the international haematological community, to assess patient need for the Soliris treatment

iv. **PHARMAC must amend its Operating Policies and Procedures** to acknowledge the right of rare disease patients to access life restoring and life saving treatments as in the specific example of the Soliris treatment

I support a Charity for a little boy named [redacted], who needs this medication. His family have dedicated their lives to fundraising for it, just so he stays alive. Every parent should be able to keep their children alive if they have a condition that medicine can treat. That a little boy, so full of potential, has his life hinging on his family and friends raising hundreds of thousands of dollars is not fair. Please consider your decision carefully.

Yours faithfully

Jen Schroder

From: [redacted]
Sent: Monday, 17 June 2013 6:30 p.m.
To: eculizumabfeedback
Subject: Submission re funding for Soliris

To Whom it May Concern;

I support PNH patients in New Zealand in their fight to gain access to the life saving treatment Soliris.

I DO NOT support PHARMAC's proposal to decline funding for the Soliris treatment.

I support the PNH Support Association's proposition for Equity and Fairness which states:

i. **PHARMAC must return to the negotiating table** with the supplier of the Soliris treatment

ii. **PHARMAC must negotiate in good faith** toward funding Soliris for a minimum of 8 NZ PNH patients

iii. **PHARMAC must establish fair assessment criteria**, based on expert advice from the international haematological community, to assess patient need for the Soliris treatment

iv. **PHARMAC must amend its Operating Policies and Procedures** to acknowledge the right of rare disease patients to access life restoring and life saving treatments as in the specific example of the Soliris treatment

I have a friend who for many years has been in and out of hospital with her PNH. With the life-saving treatment Soliris her quality of life and life expectancy would be greatly enhanced and increased. There is no alternative to this treatment and I urge PHARMAC to reconsider its decision to decline funding for the small number of New Zealand PNH patients who so desperately need it.

Yours faithfully

[redacted]

From: Jessica Copping [redacted]
Sent: Friday, 31 May 2013 9:43 p.m.
To: eculizumabfeedback
Subject: Submission to PHARMAC's Proposal to decline a funding application for Soliris (eculizumab)

To Whom it May Concern;

I support PNH patients in New Zealand in their fight to gain access to the life saving treatment Soliris.

I DO NOT support PHARMAC's proposal to decline funding for the Soliris treatment.

I support the PNH Support Association's proposition for Equity and Fairness which states:

i. **PHARMAC must return to the negotiating table** with the supplier of the Soliris treatment

ii. **PHARMAC must negotiate in good faith** toward funding Soliris for a minimum of 8 NZ PNH patients

iii. **PHARMAC must establish fair assessment criteria**, based on expert advice from the international haematological community, to assess patient need for the Soliris treatment

iv. **PHARMAC must amend its Operating Policies and Procedures** to acknowledge the right of rare disease patients to access life restoring and life saving treatments as in the specific example of the Soliris treatment

What if it was your son, your daughter, your spouse who needed this medicine to live? Would you, could you still say no?

NZ must live up to its reputation as one of the best countries in the world to live in. For that, its people need to live. Some of them need expensive medicine for that. That is not their fault. We need to support them as a community. They don't want to live on medication for the rest of their lives; they're not trying to scam the system. It's not a lifestyle choice, it's not the result of a mistake they have made. Whether it's an 'Act of God' or a simple biological misfortune; if we can help them, then we should.

Thanking you for your time and reconsideration.

Sincerely,

Jessica Copping

From: Jessica Orpana [redacted]
Sent: Tuesday, 11 June 2013 8:30 p.m.
To: eculizumabfeedback
Subject: Need the drug to save lives

To Whom it May Concern;

I support PNH patients in New Zealand in their fight to gain access to the life saving treatment Soliris.

I DO NOT support PHARMAC's proposal to decline funding for the Soliris treatment.

I support the PNH Support Association's proposition for Equity and Fairness which states:

1. **PHARMAC must return to the negotiating table** with the supplier of the Soliris treatment
2. **PHARMAC must negotiate in good faith** toward funding Soliris for a minimum of 8 NZ PNH patients
3. **PHARMAC must establish fair assessment criteria**, based on expert advice from the international haematological community, to assess patient need for the Soliris treatment
4. **PHARMAC must amend its Operating Policies and Procedures** to acknowledge the right of rare disease patients to access life restoring and life saving treatments as in the specific example of the Soliris treatment

Everybody has a right to medical help, we live in a country that should be supporting this drug for those in need.

Yours faithfully

Jessica Orpana

From: Jess Were [redacted]
Sent: Monday, 3 June 2013 10:54 a.m.
To: eculizumabfeedback
Subject: Pnh

Hi there,

I am just trying to do my part to help support those people living with the condition Pnh in NZ and require Solaris to keep on living, please reconsider funding this drug in nz!

We might be a small country but there is a lot of us living with horrible conditions and it feels like the government doesn't allow Swift access to cheap Health care or even treatment in the case of those with Pnh.

Thank you for your time

Jessica Were

From: Jethro Morrow [redacted]
Sent: Tuesday, 30 July 2013 7:27 p.m.
To: eculizumabfeedback
Subject: Submission to PHARAMC'S proposal to decline a funding application for Soliris (eculizumab)

To whom it may concern:
I support the PNH patients in New Zealand in their fight to gain access to the lifesaving treatment Soliris.
I DO NOT support Pharmac's proposed intent to decline this treatment When Pharmac take this type of consultation to the public I should be able to rely on Pharmac to provide accurate and reliable information that will help guide responses from the community. In this consultation you have overstated the number of people affected with PNH which in turn over exaggerates the likely real cost of treatment.
You did this back in 2011 when Pharmac staff reported to the Board that there would be up to 100 affected patients in New Zealand with Pompe disease. That treatment was declined, and it looks as though you intend to decline Soliris, again with dubious data as the basis for a decision. This is not acceptable and you should withdraw this consultation because of the misleading information in it, which is likely to skew responses from the public. .

These patients have a right to life. Our health system has a duty to address their needs in a fair and equitable way. When Pharmac places so much emphasis on costs, cost-effectiveness and alternative use of the money, but does not address issues of rights, equity, fairness and community values, you are failing in your duty. The District Health Boards have this duty, and Pharmac is acting as their purchasing agency. You should use the same decision criteria and priorities that they have.

Patients with rare diseases are disadvantaged because their condition is rare, and when treatments become available they are doubly affected by the high cost and the very small market due to small numbers. It is a denial of their right to health, and contrary to goals of "Equity of access, reducing inequalities and improving health outcomes for individuals and communities will guide our relationship and decision making", which are set out in the agreement between DHBs and Pharmac about how you decide things on their behalf.

Decision Criteria: The decision criteria used to assess medicines are not fair for those who are affected by rare diseases. There should be an additional layer of decision-making for rare diseases that do not fit the standard cost effectiveness threshold for large populations.
These additional layers already exist in Australia, England, Scotland and other places around the world, because they have recognised that it's a fair way to deal with the disadvantage of rare diseases.

Pharmac's assumption that "best health outcomes" as mentioned in its legislation, can be strongly associated with calculation of Quality Adjusted Life Years, is too narrow a view of what is "best", especially when Pharmac's calculations do not take into account non-health-sector costs. The decision criteria should include a broader range of considerations that are important to patients, so that the decisions are made in a patient-centred way.
Pharmac also has a responsibility for "health outcomes that are reasonably achievable". Your narrow perspective on technical assessment and budget management, to the exclusion of patient rights and interests from decisions, are an outcome that effectively discriminates against patients with rare diseases, and is not a reasonable outcome by any measure.
Pharmac often emphasises the "tough decision" approach that some will be funded and others not. That is too simplistic and can also be unfair, as it is in this case. There are very good reasons to adopt an equitable approach that spreads medicine funding across as many areas of health need as possible so that no group is completely abandoned.
That is the approach across a wide range of services in our health system and Pharmac should adopt a similar approach, rather than exclude and abandon certain groups.
I do not support your intent to decline treatment for this group or other patients where there are therapies for rare diseases.

From: Jo G [redacted]
Sent: Friday, 31 May 2013 8:43 p.m.
To: eculizumabfeedback
Subject: Soliris Funding

To Whom it May Concern;

I support PNH patients in New Zealand in their fight to gain access to the life saving treatment Soliris.

I DO NOT support PHARMAC's proposal to decline funding for the Soliris treatment.

I support the PNH Support Association's proposition for Equity and Fairness which states:

i. **PHARMAC must return to the negotiating table** with the supplier of the Soliris treatment

ii. **PHARMAC must negotiate in good faith** toward funding Soliris for a minimum of 8 NZ PNH patients

iii. **PHARMAC must establish fair assessment criteria**, based on expert advice from the international haematological community, to assess patient need for the Soliris treatment

iv. **PHARMAC must amend its Operating Policies and Procedures** to acknowledge the right of rare disease patients to access life restoring and life saving treatments as in the specific example of the Soliris treatment

If there was only one patient in New Zealand who had a need for this medication and could not afford it, that's reason enough for it to be funded to keep that one person alive. It's not anyone's place to decide that that one person doesn't deserve their chance.

Yours faithfully

Jo Carolan
[redacted]

From: Jo Davies [redacted]
Sent: Wednesday, 31 July 2013 5:39 p.m.
To: eculizumabfeedback
Subject: Submission by Jo Davies
Attachments: Submission to PHARMAC on Soliris.docx

Please find my submission attached

Regards,

Jo Davies

To: Sue Anne Yee
Therapeutic Group Manager
PHARMAC

Email: eculizumabfeedback@pharmac.govt.nz
Fax: 04 460 4995
Post: PO Box 10 254, Wellington 6143

Submission on the proposal to decline a funding application for eculizumab.

Submitted by: Jo Davies
[redacted]

I support the PNH patients in New Zealand in their fight to gain access to the life-saving treatment Soliris.
I **DO NOT** support PHARMAC's proposed intent to decline this treatment.

- I accept that this is a very expensive treatment but do not believe that a treatment should be denied purely on a basis of cost, particularly when it is a life saving treatment. This is putting a cost on a life which is morally wrong. I believe that need should be taken into account when considering which medicines to fund within a limited budget. I do not accept that because more people can receive a less expensive medicine, that medicine should have priority in funding decisions when there may not be such a 'need' for it by the patient group, i.e. it is not life saving.

- I think it is a great shame that 40 other countries fund this treatment for the small number of patients who need it and New Zealand, again, falls far behind in the funding of new and innovative medicines. I cannot begin to imagine how let down those patients must feel. I sincerely hope that nobody I know finds themselves in this situation in the future and cannot help but wonder if PHARMAC's decision making would be different if a family member suffered from PNH. I believe we need to consider the right to life and cannot tell people that their life is not worth saving.

- I would like to see PHARMAC applying more pressure to the government to increase the pharmaceutical budget. I have read that New Zealand spends only 9.4% of its health expenditure on medicines compared to an average of 16% across OECD countries and New Zealand ranks 31st out of 32 countries in the amount spent on pharmaceuticals as a share of GDP. I know that PHARMAC advises the Health Minister / Government on the pharmaceutical budget and more needs to be done to ensure that New Zealand is not continually lagging behind in health ratings. I am aware that New Zealand currently suffers a range of important health issues, but improving access to medicines can surely decrease pressure on other areas of the health system.

- I believe that PHARMAC is not addressing the issue of equity and fairness in their decision making on Soliris. We continually hear of patients with rare conditions being denied access to medicines. I believe this is a case of discrimination against minority groups who are clearly disadvantaged. The PNH group is a very small group of patients and because of their small number, it is difficult to make their voice heard and for their situation to be understood by the general public. If a life saving medicine were being declined to a much larger group, it would be a different story, for example, in the case of Herceptin funding being pushed through, rightly so, by the public and change of government. I can imagine that there won't be a huge number of submissions on this consultation which has not been helped by the misinformation provided by PHARMAC. The number of patients with PNH has been exaggerated. I believe there is also misinformation about the cost of the medicine. There was even an attempt by Dr Peter Moodie on national television to tell the public that no other countries fund this medicine.

- This consultation should not be considered an accurate picture of public opinion because of the misleading information provided by PHARMAC.

From: Mike and Jo Grogan [redacted]
Sent: Saturday, 27 July 2013 7:21 p.m.
To: eculizumabfeedback

To Whom it May Concern;

I support PNH patients in New Zealand in their fight to gain access to the life saving treatment Soliris.

I DO NOT support PHARMAC's proposal to decline funding for the Soliris treatment.

I support the PNH Support Association's proposition for Equity and Fairness which states:

i. **PHARMAC must return to the negotiating table** with the supplier of the Soliris treatment

ii. **PHARMAC must negotiate in good faith** toward funding Soliris for a minimum of 8 NZ PNH patients

iii. **PHARMAC must establish fair assessment criteria**, based on expert advice from the international haematological community, to assess patient need for the Soliris treatment

iv. **PHARMAC must amend its Operating Policies and Procedures** to acknowledge the right of rare disease patients to access life restoring and life saving treatments as in the specific example of the Soliris treatment

(please include your personal statements here)

Yours faithfully

Jo Grogan [redacted]

From: Ben and Jo [redacted]
Sent: Wednesday, 24 July 2013 9:47 a.m.
To: eculizumabfeedback
Subject: Submission to PHARAMC'S proposal to decline a funding application for Soliris (eculizumab)

Sue Anne Yee
Therapeutic Group Manager
PHARMAC

To whom it may concern:

I support the PNH patients in New Zealand in their fight to gain access to the lifesaving treatment Soliris.

I **DO NOT** support Pharmac's proposed intent to decline this treatment.

- These patients have a right to life. Our health system has a duty to address their needs in a fair and equitable way. When Pharmac places so much emphasis on costs, cost-effectiveness and alternative use of the money, but does not address issues of rights, equity, fairness and community values; you are failing in your duty. The District Health Boards have this duty, and Pharmac is acting as their purchasing agency. You should use the same decision criteria and priorities that they have.

- Patients with rare diseases are disadvantaged because their condition is rare, and when treatments become available they are doubly affected by the high cost and the very small market due to small numbers. It is a denial of their right to health, and contrary to goals of "Equity of access, reducing inequalities and improving health outcomes for individuals and communities will guide our relationship and decision making", which are set out in the agreement between DHBs and Pharmac about how you decide things on their behalf.

- **Decision Criteria:** The decision criteria used to assess medicines are not fair for those who are affected by rare diseases. There should be an additional layer of decision-making for rare diseases that do not fit the standard cost effectiveness threshold for large populations. These additional layers already exist in Australia, England, Scotland and other places around the world, because they have recognised that it's a fair way to deal with the disadvantage of rare diseases.

- Pharmac's assumption that "best health outcomes" as mentioned in its legislation, can be strongly associated with calculation of Quality Adjusted Life Years, is too narrow a view of what is "best", especially when Pharmac's calculations do not take into account non-health-sector costs. The decision criteria should include a broader range of considerations that are important to patients, so that the decisions are made in a patient-centred way.

- Pharmac also has a responsibility for "health outcomes that are reasonably achievable". Your narrow perspective on technical assessment and budget management, to the exclusion of patient rights and interests from decisions, are an outcome that effectively discriminates against patients with rare diseases, and is not a reasonable outcome by any measure.

- Pharmac often emphasises the "tough decision" approach that some will be funded and others not. That is too simplistic and can also be unfair, as it is in this case. There are very good reasons to adopt an equitable approach that spreads medicine funding across as many areas of health need as possible, so that no group is completely abandoned. That is the approach across a wide range of services in our health system and Pharmac should adopt a similar approach, rather than exclude and abandon certain groups.

I do not support your intent to decline treatment for the PNH group or other patients where there are therapies for rare diseases.

Jo Heslop

To: eculizumabfeedback[eculizumabfeedback@Pharmac.govt.nz];
Flag Status: 0x00000000
Subject: Submission to PHARMAC's Proposal to decline a funding application for Soliris (eculizumab)
From: Jo Sutherland
Sent: Thur 6/20/2013 9:18:19

To Whom it May Concern;

I support PNH patients in New Zealand in their fight to gain access to the life saving treatment Soliris.

I DO NOT support PHARMAC's proposal to decline funding for the Soliris treatment.

I support the PNH Support Association's proposition for Equity and Fairness which states:

1. **PHARMAC must return to the negotiating table** with the supplier of the Soliris treatment
2. **PHARMAC must negotiate in good faith** toward funding Soliris for a minimum of 8 NZ PNH patients
3. **PHARMAC must establish fair assessment criteria**, based on expert advice from the international haematological community, to assess patient need for the Soliris treatment
4. **PHARMAC must amend its Operating Policies and Procedures** to acknowledge the right of rare disease patients to access life restoring and life saving treatments as in the specific example of the Soliris treatment

Imagine if it was your child. Wouldn't you do anything to make this happen?

Yours faithfully

Jo Sutherland

From: [redacted]
Sent: Wednesday, 5 June 2013 10:00 a.m.
To: eculizumabfeedback
Subject: Submission to PHARMAC's Proposal to decline a funding application for Soliris (eculizumab)
Attachments: proposition_equity_and_fairness.doc

Please refer to attachment.

To Whom it May Concern;

I support PNH patients in New Zealand in their fight to gain access to the life saving treatment Soliris.

I DO NOT support PHARMAC's proposal to decline funding for the Soliris treatment.

I support the PNH Support Association's proposition for Equity and Fairness which states:

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ii. **PHARMAC must negotiate in good faith** toward funding Soliris for a minimum of 8 NZ PNH patients

iii. **PHARMAC must establish fair assessment criteria**, based on expert advice from the international haematological community, to assess patient need for the Soliris treatment

iv. **PHARMAC must amend its Operating Policies and Procedures** to acknowledge the right of rare disease patients to access life restoring and life saving treatments as in the specific example of the Soliris treatment

I am personally lucky enough not to need this drug to stay alive. My good friends young son however is not. [redacted] *who is almost* [redacted] *years old will inevitably depend on Soliris to live. The quality of life this little survivor has been able to experience has been restricted by a life lived in hospital attached to machines. The cost of his infusions of fresh frozen plasma amongst other treatments must surely be costing more than the cost for him to be on Soliris. The potential life this amazing child could live if this drug is funded for him will be a brilliant one. Where is the social justice??? Who thinks they have the power to give or revoke life? This child was not born into poverty; he was born into a hard working middle class family. This family have had to give up so much just to care for him and be there for him while he goes through his regular hospital treatment and in the meantime are vigorously fundraising in an attempt to reach the annual bounty that PHARMAC has put on this young life. I ask you, if this was your child or loved one and you had the power to fix this situation, would there be a price for their life too? Surely common-sense would prevail and the realisation that the long term cost of Jethro's current treatment regime would equate to a similar amount to that of the half a million annual fee to have him on Soliris? In the meantime, a hospital bed could be freed up for another sick child. Support could be given to another family in need and social justice could prevail.*

Yours faithfully

[redacted]

From: OPP Review
Sent: Wednesday, 5 June 2013 10:52 a.m.
To: eculizumabfeedback
Subject: FW: Submission in response to PHARMAC's consultation on Decision Critera.Submission in response to PHARMAC's consultation on Decision Critera.

[SEEMail]

From: Jo O'Boyle [redacted]
Sent: Sunday, 2 June 2013 10:14 a.m.
To: OPP Review
Subject: Submission in response to PHARMAC's consultation on Decision Critera.Submission in response to PHARMAC's consultation on Decision Critera.

To Whom It May Concern;

I support PNH patients in New Zealand in their fight to gain access to the life saving treatment Soliris.

I DO NOT support PHARMAC's proposal to decline funding for the Soliris treatment.

I support the PNH Support Association's proposition for Equity and Fairness which states:

i. **PHARMAC must return to the negotiating table** with the supplier of the Soliris treatment

ii. **PHARMAC must negotiate in good faith** toward funding Soliris for a minimum of 8 NZ PNH patients

iii. **PHARMAC must establish fair assessment criteria**, based on expert advice from the international haematological community, to assess patient need for the Soliris treatment

iv. **PHARMAC must amend its Operating Policies and Procedures** to acknowledge the right of rare disease patients to access life restoring and life saving treatments as in the specific example of the Soliris treatment

Yours faithfully

Joanna O'Boyle

To Whom It May Concern;

I support PNH patients in New Zealand in their fight to gain access to the life saving treatment Soliris.

I DO NOT support PHARMAC's proposal to decline funding for the Soliris treatment.

I support the PNH Support Association's proposition for Equity and Fairness which states:

i. **PHARMAC must return to the negotiating table** with the supplier of the Soliris treatment

ii. **PHARMAC must negotiate in good faith** toward funding Soliris for a minimum of 8 NZ PNH patients

iii. **PHARMAC must establish fair assessment criteria**, based on expert advice from the international haematological community, to assess patient need for the Soliris treatment

iv. **PHARMAC must amend its Operating Policies and Procedures** to acknowledge the right of rare disease patients to access life restoring and life saving treatments as in the specific example of the Soliris treatment

Yours faithfully

Joanna O'Boyle

From: [redacted]
Sent: Monday, 3 June 2013 6:57 a.m.
To: eculizumabfeedback
Subject: Submission to PHARMAC's Proposal to decline a funding

To Whom it May Concern;

I support PNH patients in New Zealand in their fight to gain access to the life saving treatment Soliris.

I DO NOT support PHARMAC's proposal to decline funding for the Soliris treatment.

I support the PNH Support Association's proposition for Equity and Fairness which states:

i. **PHARMAC must return to the negotiating table** with the supplier of the Soliris treatment

ii. **PHARMAC must negotiate in good faith** toward funding Soliris for a minimum of 8 NZ PNH patients

iii. **PHARMAC must establish fair assessment criteria,** based on expert advice from the international haematological community, to assess patient need for the Soliris treatment

iv. **PHARMAC must amend its Operating Policies and Procedures** to acknowledge the right of rare disease patients to access life restoring and life saving treatments as in the specific example of the Soliris treatment

I have a friend who's brother is largely affected by this and if he cannot get funded for this drug, it will significantly reduce his life expectancy.

Yours faithfully

[redacted]

From: [redacted]
Sent: Thursday, 30 May 2013 8:48 a.m.
To: eculizumabfeedback
Subject: Submission to PHARMAC's Proposal to decline a funding application for Soliris (eculizumab)

To Whom it May Concern;

I support PNH patients in New Zealand in their fight to gain access to the life saving treatment Soliris.

I DO NOT support PHARMAC's proposal to decline funding for the Soliris treatment.

I support the PNH Support Association's proposition for Equity and Fairness which states:

i. **PHARMAC must return to the negotiating table** with the supplier of the Soliris treatment

ii. **PHARMAC must negotiate in good faith** toward funding Soliris for a minimum of 8 NZ PNH patients

iii. **PHARMAC must establish fair assessment criteria**, based on expert advice from the international haematological community, to assess patient need for the Soliris treatment

iv. **PHARMAC must amend its Operating Policies and Procedures** to acknowledge the right of rare disease patients to access life restoring and life saving treatments as in the specific example of the Soliris treatment

This treatment can save my brothers life, we need PHARMAC to do the right thing and fund Soliris. I want my children to grow up in a country that provides fair health care to all New Zealanders based on fair assessment of need and results.

The facts provided in the reason to decline are simply un true. It is disgusting that they can get away this, it is a life saving treatment that works.

Yours faithfully

[redacted]

From: Val devlin [redacted]
Sent: Monday, 29 July 2013 5:28 p.m.
To: eculizumabfeedback
Subject: Soliris Treatment funding

To Whom it May Concern;

I support PNH patients in New Zealand in their fight to gain access to the life saving treatment Soliris.
I DO NOT support PHARMAC's proposal to decline funding for the Soliris treatment.
I support the PNH Support Association's proposition for Equity and Fairness which states:

i. **PHARMAC must return to the negotiating table** with the supplier of the Soliris treatment
ii. **PHARMAC must negotiate in good faith** toward funding Soliris for a minimum of 8 NZ PNH patients
iii. **PHARMAC must establish fair assessment criteria**, based on expert advice from the international haematological community, to assess patient need for the Soliris treatment
iv. **PHARMAC must amend its Operating Policies and Procedures** to acknowledge the right of rare disease patients to access life restoring and life saving treatments as in the specific example of the Soliris treatment

Please reconsider funding Soliris for our NZ sufferers.

Yours faithfully,

Joe and Valerie Devlin,
[redacted]

From: Joe [redacted]
Sent: Tuesday, 25 June 2013 9:46 a.m.
To: eculizumabfeedback
Subject: allow funding

expense should be the last thing that matters. what comes first is the improvement of quality of life.
I submit that this should be funded.

From: borse25 [redacted]
Sent: Monday, 17 June 2013 1:13 a.m.
To: eculizumabfeedback
Subject: Soliris

Dear Sir This drug should be funded by Pharmac to improve the quality of life of those unfortunate New Zealanders suffering from PNH. They all deserve a chance at a life that will offer hope and be able to contribute to this country..please give them a shot at a rosier future.Regards John Garner.

Sent from my phone on the smartphonetwork.

From: John Vosper [redacted]
Sent: Wednesday, 17 July 2013 9:25 a.m.
To: eculizumabfeedback; OPP Review
Subject: Re: Submission opposing PHARMAC's Proposal to decline a funding application for Soliris (eculizumab)

Re: Submission opposing PHARMAC's Proposal to decline a funding application for Soliris (eculizumab)

To Whom it May Concern;

I SUPPORT PNH patients in New Zealand in their fight to gain access to the lifesaving treatment Soliris.

I OPPOSE PHARMAC's proposal to decline funding for the Soliris treatment.

I support the PNH Support Association's proposition for Equity and Fairness which states:

1. **PHARMAC must return to the negotiating table** with the supplier of the Soliris treatment
2. **PHARMAC must negotiate in good faith** toward funding Soliris for a minimum of 8 NZ PNH patients
3. **PHARMAC must establish fair assessment criteria**, based on expert advice from the international haematological community, to assess patient need for the Soliris treatment
4. **PHARMAC must amend its Operating Policies and Procedures** to acknowledge the right of rare disease patients to access life restoring and lifesaving treatments as in the specific example of the Soliris treatment

I could understand if there were alternative treatments that were as effective but as there are not this is a grossly unfair decision. This disease is not as a result of life style choices and a treatment should be funded

Yours faithfully,
John Vosper

[redacted]

From: Johnny Lane [redacted]
Sent: Wednesday, 31 July 2013 11:03 p.m.
To: eculizumabfeedback
Subject: Submission to PHARAMC'S proposal to decline a funding application for Soliris (eculizumab)

Sue Anne Yee

I support the PNH patients in New Zealand in their fight to gain access to the lifesaving treatment Soliris.
I **DO NOT** support Pharmac's proposed intent to decline this treatment.

- When Pharamac takes this type of consultation to the public I should be able to rely on Pharmac to provide accurate and reliable information that will help guide responses from the community. In this consultation you have overstated the number of people affected with PNH which in turn over exaggerates the likely real cost of treatment. You did this back in 2011 when Pharmac staff reported to the Board that there would be up to 100 affected patients in New Zealand with Pompe disease. That treatment was declined, and it looks as though you intend to decline Soliris, again with dubious data as the basis for a decision. This is not acceptable and you should withdraw this consultation because of the misleading information in it, which is likely to skew responses from the public.

- These patients have a right to life. Our health system has a duty to address their needs in a fair and equitable way. When Pharmac places so much emphasis on costs, cost-effectiveness and alternative use of the money, but does not address issues of rights, equity, fairness and community values; you are failing in your duty. The District Health Boards have this duty, and Pharmac is acting as their purchasing agency. You should use the same decision criteria and priorities that they have.

- Patients with rare diseases are disadvantaged because their condition is rare, and when treatments become available they are doubly affected by the high cost and the very small market due to small numbers. It is a denial of their right to health, and contrary to goals of "Equity of access, reducing inequalities and improving health outcomes for individuals and communities will guide our relationship and decision making", which are set out in the agreement between DHBs and Pharmac about how you decide things on their behalf.

- **Decision Criteria:** The decision criteria used to assess medicines are not fair for those who are affected by rare diseases. There should be an additional layer of decision-making for rare diseases that do not fit the standard cost effectiveness threshold for large populations. These additional layers already exist in Australia, England, Scotland and other places around the world, because they have recognised that it's a fair way to deal with the disadvantage of rare diseases.

- Pharmac's assumption that "best health outcomes" as mentioned in its legislation, can be strongly associated with calculation of Quality Adjusted Life Years, is too narrow a view of what is "best", especially when Pharmac's calculations do not take into account non-health-sector costs. The decision criteria should include a broader range of considerations that are important to patients, so that the decisions are made in a patient-centred way.

- Pharmac also has a responsibility for "health outcomes that are reasonably achievable". Your narrow perspective on technical assessment and budget management, to the exclusion of patient rights and interests from decisions, are an outcome that effectively discriminates against patients with rare diseases, and is not a reasonable outcome by any measure.

- Pharmac often emphasises the "tough decision" approach that some will be funded and others not. That is too simplistic and can also be unfair, as it is in this case. There are very good reasons to adopt an equitable approach that spreads medicine funding across as many areas of health need as possible, so that no group is completely abandoned. That is the approach across a wide range of services in our health system and Pharmac should adopt a similar approach, rather than exclude and abandon certain groups.

I do not support your intent to decline treatment for the PNH group or other patients where there are therapies for rare diseases.

Yours sincerely

Johnny Lane

From: Johnny Stevenson [redacted]
Sent: Monday, 29 July 2013 9:28 a.m.
To: eculizumabfeedback
Subject: Submission to PHARAMC'S proposal to decline a funding application for Soliris (eculizumab)
Attachments: 20130729093032747.pdf

Johnny Stevenson
[redacted]

To provide feedback, please submit it in writing by **Wednesday, 31 July 2013** to:

Sue Anne Yee
Therapeutic Group Manager
PHARMAC

Email: eculizumabfeedback@pharmac.govt.nz
Fax: 04 460 4995
Post: PO Box 10 254, Wellington 6143

Subject: **Submission to PHARAMC'S proposal to decline a funding application for Soliris (eculizumab)**

To whom it may concern:

I support the PNH patients in New Zealand in their fight to gain access to the lifesaving treatment Soliris.

I **DO NOT** support Pharmac's proposed intent to decline this treatment.

- When Pharamc takes this type of consultation to the public I should be able to rely on Pharmac to provide accurate and reliable information that will help guide responses from the community. In this consultation you have overstated the number of people affected with PNH which in turn over exaggerates the likely real cost of treatment. You did this back in 2011 when Pharmac staff reported to the Board that there would be up to 100 affected patients in New Zealand with Pompe disease. That treatment was declined, and it looks as though you intend to decline Soliris, again with dubious data as the basis for a decision. This is not acceptable and you should withdraw this consultation because of the misleading information in it, which is likely to skew responses from the public.

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- Patients with rare diseases are disadvantaged because their condition is rare, and when treatments become available they are doubly affected by the high cost and the very small market due to small numbers. It is a denial of their right to health, and contrary to goals of "Equity of access, reducing inequalities and improving health outcomes for individuals and communities will guide our relationship and decision making", which are set out in the agreement between DHBs and Pharmac about how you decide things on their behalf.

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- Pharmac often emphasises the "tough decision" approach that some will be funded and others not. That is too simplistic and can also be unfair, as it is in this case. There are very good reasons to adopt an equitable approach that spreads medicine funding across as many areas of health need as possible, so that no group is completely abandoned. That is the approach across a wide range of services in our health system and Pharmac should adopt a similar approach, rather than exclude and abandon certain groups.

I do not support your intent to decline treatment for the PNH group or other patients where there are therapies for rare diseases.

30/09/13

From: Jon coley [redacted]
Sent: Saturday, 20 July 2013 3:14 a.m.
To: eculizumabfeedback
Subject: Soliris

Please reconsider your decision concerning Soliris. Lives of precious people depend on this decision.
Sent from my iPhone

From: Jonny McEwan [redacted]
Sent: Tuesday, 11 June 2013 5:57 p.m.
To: eculizumabfeedback
Subject: Submission to PHARMAC's Proposal to decline a funding application for Soliris (eculizumab)

To Whom it May Concern;

I support PNH patients in New Zealand in their fight to gain access to the life saving treatment Soliris.

I DO NOT support PHARMAC's proposal to decline funding for the Soliris treatment.

I support the PNH Support Association's proposition for Equity and Fairness which states:

i. **PHARMAC must return to the negotiating table** with the supplier of the Soliris treatment

ii. **PHARMAC must negotiate in good faith** toward funding Soliris for a minimum of 8 NZ PNH patients

iii. **PHARMAC must establish fair assessment criteria,** based on expert advice from the international haematological community, to assess patient need for the Soliris treatment

iv. **PHARMAC must amend its Operating Policies and Procedures** to acknowledge the right of rare disease patients to access life restoring and life saving treatments as in the specific example of the Soliris treatment

Yours faithfully

Jonathan McEwan

From: [redacted]
Sent: Wednesday, 24 July 2013 3:34 a.m.
To: eculizumabfeedback
Subject: Submission to PHARMAC's Proposal to decline a funding application for Soliris (eculizumab)

To Whom it May Concern;

My sister-in-law, [redacted]*, suffers from PNH and while I am not a permanent resident of New Zealand I find it unacceptable that her country is not providing her the option to have access to the treatment her doctor's prescribed. This is not an issue of cost, but an issue of life and as a first world society, which I do believe New Zealand is, it is the government's responsibility to its citizens to provide the care and infrastructure they need to lead healthy and productive lives. In choosing to not fund Soliris you are choosing to not provide that care and infrastructure to some of your citizens. While you may argue this only affects a small few, I suggest that you are now treading down the slippery slope of not providing care because it's an inconvenience or considered to be too great an expense. It is not your choice to determine which patients or which citizens get to lead healthy and productive lives and which patients/citizens get to suffer. I strongly urge you to reconsider your position on Soliris and provide the care and treatment that your citizens need, I know I would expect the same from my country.*

I support PNH patients in New Zealand in their fight to gain access to the life saving treatment Soliris.

I DO NOT support PHARMAC's proposal to decline funding for the Soliris treatment.

I support the PNH Support Association's proposition for Equity and Fairness which states:

i. **PHARMAC must return to the negotiating table with the supplier of the Soliris treatment**

ii. **PHARMAC must negotiate in good faith toward funding Soliris for a minimum of 8 NZ PNH patients**

iii. **PHARMAC must establish fair assessment criteria, based on expert advice from the international haematological community, to assess patient need for the Soliris treatment**

iv. **PHARMAC must amend its Operating Policies and Procedures to acknowledge the right of rare disease patients to access life restoring and life saving treatments as in the specific example of the Soliris treatment**

Yours faithfully,

[redacted]

From: [redacted]
Sent: Tuesday, 30 July 2013 10:39 p.m.
To: eculizumabfeedback
Subject: Soliris funding feedback
Attachments: 2013 07 Submission to Pharmac July 2013 Julie.docx

Please find attached submission. Please note this is a second submission. First email from me contained feedback from my mother.

Submission to Pharmac regarding Soliris treatment for PNH patients

My mum has PNH. [redacted]

Since then she has been fighting PNH daily to lead as normal life as possible, [redacted] Her valuable contribution to society [redacted] is truly admirable. Would you be able to [redacted] That is what my mum has done.

[redacted]

Pharmacs decision not to fund Soliris is devastating for our family. The thought that mum has worked so hard all her life despite her illness, to provide such a wonderful upbringing for us kids, and contributed so much to NZ as a tax payer, to have her chance to lead a life she has only dreamed about snatched away by Pharmac because of money is truly cruel.

It leaves us disillusioned about the morals and values of the decision makers at Pharmac when the most important aspect of their decision making criteria appears to be money and leave compassion and respect for the preservation of human life out of the equation when deciding what treatments to provide for patients. It is so ironic that as a society people can be on their death beds and still we revive them no matter how much they may suffer until they die, and euthanasia is illegal, but when we have the chance to take away the suffering and premature death of PNH sufferers, we can't do it because Pharmac have decided that it costs too much to save these people's lives.

I implore you Pharmac to reverse your decision not to fund Soliris, the ONLY effective treatment for PNH patients. The ability of this treatment to eliminate symptoms for PNH sufferers and extend their life should be reason enough to fund it. Pharmacs treatment of PNH patients by their refusal to fund Soliris can be likened to that of how you would look at a sick cow. She costs too much to save so we will "cull" her. However we are not dealing with cows here, but humans. We cannot put a price on a human life and it is time that Pharmac acknowledges this and starts saving PNH patients lives!

From: Julie Wells [redacted]
Sent: Sunday, 28 July 2013 8:16 p.m.
To: eculizumabfeedback
Subject: Submission to PHARAMC'S proposal to decline a funding application for Soliris (eculizumab)

To whom it may concern:
I support the PNH patients in New Zealand in their fight to gain access to the lifesaving treatment Soliris.
I DO NOT support Pharmac's proposed intent to decline this treatment. I also believe it would be a backward step for New Zealand as a whole.
New Zealand is a civilized democracy. We are part of the United Nations and pride ourselves on out human rights record. We hold our own on the world stage in every area of excellence in spite of being a small population. We have earned respect! Our public health system and our medicines purchasing agency (Pharmac), are held up as models by which other nations strive to aspire. However, on the issue of treatment for rare diseases, these other nations take the lead while we lethargically drag our heels. We are told by Pharmac that the issue is about cost and cost effectiveness, and there is no doubt that the new, innovative medicines are costly, not just to New Zealand, but to the international market. However, in spite of these high costs, other cash strapped nations appear to be meeting the challenge. This, in itself, makes our refusal to treat even more difficult to justify.
If we take the upholding of human rights seriously, we would agree that New Zealand citizens have a right to life. Hence, we have a moral obligation to ensure that everyone has access to life sustaining treatment when such treatments become available. No clinical population should ever be completely abandoned in our society. The notion that patients with rare diseases can be ignored because the costs are higher than average, is callous and inhumane. I recognize that it must be difficult at times to balance the books, but it would be morally wrong to achieve this by denying treatment to this small, select group of patients.
Patients with rare diseases are already disadvantaged because of deteriorating health and all that this entails; they should not be subjected to the added pressures of having to fight for the health benefits that most other New Zealanders take for granted.
As I see it, a far more compassionate approach would be for Pharmac to negotiate lower prices with drug companies, which incidentally is the precise expertise upon which Pharmac has built its international reputation.
I do not support your intent to decline treatment for the PNH group or other patients where there are therapies for rare diseases.

From: Juliet Cavanagh- Eyre [redacted]
Sent: Thursday, 6 June 2013 9:35 a.m.
To: eculizumabfeedback
Subject: Submission to PHARMAC's Proposal to decline a funding application for Soliris (eculizumab)

To Whom it May Concern;

I support PNH patients in New Zealand in their fight to gain access to the life saving treatment Soliris.

I DO NOT support PHARMAC's proposal to decline funding for the Soliris treatment.

I support the PNH Support Association's proposition for Equity and Fairness which states:

i. **PHARMAC must return to the negotiating table** with the supplier of the Soliris treatment

ii. **PHARMAC must negotiate in good faith** toward funding Soliris for a minimum of 8 NZ PNH patients

iii. **PHARMAC must establish fair assessment criteria**, based on expert advice from the international haematological community, to assess patient need for the Soliris treatment

iv. **PHARMAC must amend its Operating Policies and Procedures** to acknowledge the right of rare disease patients to access life restoring and life saving treatments as in the specific example of the Soliris treatment

Yours Faithfully
Juliet Cavanagh-Eyre

From: lettie [redacted]
Sent: Thursday, 4 July 2013 9:31 p.m.
To: eculizumabfeedback
Subject: Submission to PHARMAC's Proposal to decline a funding application for Soliris (eculizumab)

To Whom it May Concern;

I support PNH patients in New Zealand in their fight to gain access to the life saving treatment Soliris.

I DO NOT support PHARMAC's proposal to decline funding for the Soliris treatment.

I support the PNH Support Association's proposition for Equity and Fairness which states:

i. **PHARMAC must return to the negotiating table** with the supplier of the Soliris treatment

ii. **PHARMAC must negotiate in good faith** toward funding Soliris for a minimum of 8 NZ PNH patients

iii. **PHARMAC must establish fair assessment criteria**, based on expert advice from the international haematological community, to assess patient need for the Soliris treatment

iv. **PHARMAC must amend its Operating Policies and Procedures** to acknowledge the right of rare disease patients to access life restoring and life saving treatments as in the specific example of the Soliris treatment

I can't believe that in a country like New Zealand we are not able to access this life saving medication without ridiculous costs

Yours faithfully

Juliette Bright

From:	Karen Murray [redacted]
Sent:	Wednesday, 5 June 2013 8:28 a.m.
To:	eculizumabfeedback
Subject:	proposition_equity_and_fairness_-_email_submission_template
Attachments:	proposition_equity_and_fairness_-_email_submission_template.doc

Please email *BOTH*

Subject: Submission to PHARMAC's Proposal to decline a funding application for Soliris (eculizumab)
Email: eculizumabfeedback@pharmac.govt.nz

AND

Subject: Submission in response to PHARMAC's consultation on Decision Critera.
Email: opp@pharmac.govt.nz

To Whom it May Concern;

I support PNH patients in New Zealand in their fight to gain access to the life saving treatment Soliris.

I DO NOT support PHARMAC's proposal to decline funding for the Soliris treatment.

I support the PNH Support Association's proposition for Equity and Fairness which states:

i. **PHARMAC must return to the negotiating table** with the supplier of the Soliris treatment

ii. **PHARMAC must negotiate in good faith** toward funding Soliris for a minimum of 8 NZ PNH patients

iii. **PHARMAC must establish fair assessment criteria**, based on expert advice from the international haematological community, to assess patient need for the Soliris treatment

iv. **PHARMAC must amend its Operating Policies and Procedures** to acknowledge the right of rare disease patients to access life restoring and life saving treatments as in the specific example of the Soliris treatment

Yours faithfully

Karen Murray

[redacted]

From: Katherine Heath [redacted]
Sent: Sunday, 2 June 2013 12:45 a.m.
To: eculizumabfeedback
Subject: Submission to PHARMAC's Proposal to Decline a Funding Application for Soliris (Eculizumab)

To Whom it May Concern;

I support PNH patients in New Zealand in their fight to gain access to the life saving treatment Soliris.

I DO NOT support PHARMAC's proposal to decline funding for the Soliris treatment.

I support the PNH Support Association's proposition for Equity and Fairness which states:

i. **PHARMAC must return to the negotiating table** with the supplier of the Soliris treatment

ii. **PHARMAC must negotiate in good faith** toward funding Soliris for a minimum of 8 NZ PNH patients

iii. **PHARMAC must establish fair assessment criteria**, based on expert advice from the international haematological community, to assess patient need for the Soliris treatment

iv. **PHARMAC must amend its Operating Policies and Procedures** to acknowledge the right of rare disease patients to access life restoring and life saving treatments as in the specific example of the Soliris treatment

Yours Faithfully
Katherine Heath

From: Katherine Lim [redacted]
Sent: Wednesday, 5 June 2013 12:47 p.m.
To: eculizumabfeedback
Attachments: proposition_equity_and_fairness_-_email_submission_template-1.doc

Please read the attachment.

Yours sincerely
K Lim

Please email *BOTH*

Subject: Submission to PHARMAC's Proposal to decline a funding application for Soliris (eculizumab)
Email: eculizumabfeedback@pharmac.govt.nz

AND

Subject: Submission in response to PHARMAC's consultation on Decision Critera.
Email: opp@pharmac.govt.nz

To Whom it May Concern;

I support PNH patients in New Zealand in their fight to gain access to the life saving treatment Soliris.

I DO NOT support PHARMAC's proposal to decline funding for the Soliris treatment.

I support the PNH Support Association's proposition for Equity and Fairness which states:

i. **PHARMAC must return to the negotiating table** with the supplier of the Soliris treatment

ii. **PHARMAC must negotiate in good faith** toward funding Soliris for a minimum of 8 NZ PNH patients

iii. **PHARMAC must establish fair assessment criteria**, based on expert advice from the international haematological community, to assess patient need for the Soliris treatment

iv. **PHARMAC must amend its Operating Policies and Procedures** to acknowledge the right of rare disease patients to access life restoring and life saving treatments as in the specific example of the Soliris treatment

(please include your personal statements here)

Yours faithfully

From: KATIE O'NEILL [redacted]
Sent: Thursday, 6 June 2013 12:46 p.m.
To: eculizumabfeedback
Subject: Submission to PHARMAC's Proposal to decline a funding application for Soliris (eculizumab)

To Whom it May Concern;

I support PNH patients in New Zealand in their fight to gain access to the life saving treatment Soliris.

I DO NOT support PHARMAC's proposal to decline funding for the Soliris treatment.

I support the PNH Support Association's proposition for Equity and Fairness which states:

i. **PHARMAC must return to the negotiating table** with the supplier of the Soliris treatment

ii. **PHARMAC must negotiate in good faith** toward funding Soliris for a minimum of 8 NZ PNH patients

iii. **PHARMAC must establish fair assessment criteria**, based on expert advice from the international haematological community, to assess patient need for the Soliris treatment

iv. **PHARMAC must amend its Operating Policies and Procedures** to acknowledge the right of rare disease patients to access life restoring and life saving treatments as in the specific example of the Soliris treatment

Yours faithfully

Katie O'Neill

From: [redacted]
Sent: Tuesday, 30 July 2013 8:50 p.m.
To: eculizumabfeedback
Subject: Submission - PHARMAC's Proposal to Decline Funding for Eculizumab
Attachments: PHARMAC Submission (1).docx

Attached is my submission to the above proposal.

[redacted]

SUBMISSION FOR PHARMAC TO FUND THE DRUG ECULIZUMAB (SOLIRIS) FOR THE TREATMENT OF PNH

My sister has PNH. She is [redacted] years old and works full time in [redacted] [redacted] [redacted]. Her name is [redacted]

I am writing this Submission because:

From my own point of view:

- I could not bear to be without her as a consequence of this terrible disease [redacted]
- I do not want to see my Mum & Dad go through the pain [redacted]
- I want to stop worrying about her

For [redacted]:

- I want to take away her pain
- I want to stop the worry of possible strokes and other nasties happening during periods of haemolysis activity
- I want to take away the worry of her being around others with transferable illnesses like a cold or the flu (her immunity is compromised)
- I want to remove the fatigue she lives with every day
- I want her to have a normal life expectancy
- I want her to be able to enjoy being in the sunshine (she is particularly vulnerable here)
- I want her to be free from repeated rounds of steroids and the on-going need to take warfarin
- Maybe she could even have a child

Hopefully the list above outlines some of the difficulties not only [redacted], but all PNH sufferers live with. The effects of the disease do not stop there, each of these people has a family who are affected too. It is very difficult to watch a loved one suffer and slowly deteriorate, particularly when you know there is a drug available to change their lives for the better. **But it is just out of reach!**

I cannot comprehend that PHARMAC refuses to pay for a drug that is not only life-changing/saving but gives PNH sufferers the ability to live where they belong - in New Zealand, with their friends and family. The alternative (moving to Australia, away from friends and family) is totally unacceptable, it simply screams – we just don't want you in NZ, if you do not leave, then you are effectively being given a death sentence. Sounds pretty dramatic, but that's the reality.

Budgets don't belong in this equation, how do you put a monetary amount on life?? If PHARMAC thinks the answer is in their bottom line, then their bottom line is inadequate.

[redacted] has a right to life in her country of birth just as I do. She works just as I do, she is qualified in her area of expertise just as I am, she contributes to society no less than me. Why should she be punished just because she has the misfortune to contract a rare disease.

Mum has told me about a quote made by John Key on Q & A that goes along the lines of...... "I say that one should judge a society by how it looks after it's sick and vulnerable".

As I see it, the responsibility to make this happen lies with PHARMAC. It is your responsibility to remind John Key that what he said equates to a commitment and he needs to ensure the budget is sufficient to meet that commitment.

I leave it with PHARMAC with the expectation of a turn-around........ (please).

[redacted]

From: Katrina Pace [redacted]
Sent: Thursday, 6 June 2013 4:34 p.m.
To: eculizumabfeedback
Subject: proposal to decline funding for solaris feedback

To Whom it May Concern;

I support PNH patients in New Zealand in their fight to gain access to the life saving treatment Soliris.

I DO NOT support PHARMAC's proposal to decline funding for the Soliris treatment.

I support the PNH Support Association's proposition for Equity and Fairness which states;

i. **PHARMAC must return to the negotiating table** with the supplier of the Soliris treatment

ii. **PHARMAC must negotiate in good faith** toward funding Soliris for a minimum of 8 NZ PNH patients

iii. **PHARMAC must establish fair assessment criteria**, based on expert advice from the international haematological community, to assess patient need for the Soliris treatment

iv. **PHARMAC must amend its Operating Policies and Procedures** to acknowledge the right of rare disease patients to access life restoring and life saving treatments as in the specific example of the Soliris treatment

Yours faithfully

Katrina Pace

Katrina Pace [redacted]

From: [redacted]
Sent: Sunday, 9 June 2013 3:22 p.m.
To: eculizumabfeedback
Cc: OPP Review
Subject: Emailing: [redacted] SUBMISSION RE PHARMAC'S PROPOSAL TO DECLINE FUNDING FOR SOLIRIS (ECULIZUMAB)
Attachments: [redacted] SUBMISSION RE PHARMAC'S PROPOSAL TO DECLINE FUNDING FOR SOLIRIS (ECULIZUMAB).pdf

[redacted] SUBMISSION RE PHARMAC'S PROPOSAL TO DECLINE FUNDING FOR SOLIRIS (ECULIZUMAB)

To Whom it may Concern,

Please find or submission attached.

Regards [redacted]

[redacted]

To Whom it May Concern;

I support PNH patients in New Zealand in their fight to gain access to the life saving treatment Soliris.

I DO NOT support PHARMAC's proposal to decline funding for the Soliris treatment.

I support the PNH Support Association's proposition for Equity and Fairness which states:

i. **PHARMAC must return to the negotiating table** with the supplier of the Soliris treatment

ii. **PHARMAC must negotiate in good faith** toward funding Soliris for a minimum of 8 NZ PNH patients

iii. **PHARMAC must establish fair assessment criteria,** based on expert advice from the international haematological community, to assess patient need for the Soliris treatment

iv. **PHARMAC must amend its Operating Policies and Procedures** to acknowledge the right of rare disease patients to access life restoring and life saving treatments as in the specific example of the Soliris treatment

We are the parents of [redacted] and who also suffers from PNH. We have seen the struggles [redacted] while coping with low energy levels due to PNH. We are also aware that he faces an uncertain future.

Near the end of [redacted] which could have taken his life. We need to fund Soliris.

I was very disappointed when I saw Pharmac **exaggerated** the cost of the drug at $600,000 (Peter Moody never queried the lower price mentioned when interviewed on TV One Morning Report early this year) and **exaggerated** the number of patients requiring Soliris. This is simply to mislead the New Zealand Public.

Clinical research has shown an increase of life span 32.5 years .The Australian Advisory Committee (PBAC) agree that the study program and dataset for eculizumab(Soliris) in PNH is the most significant, qualitative and powerful set of results produced to date for an ultra-orphan medicine for a rare life-threatening disease.

People suffer from PNH through no fault of their own. However if they were drunk and had an accident their medical care would be paid for. **IT IS TIME TO BE FAIR.**

[redacted]

From: Kelli Richards [redacted]
Sent: Saturday, 1 June 2013 8:38 a.m.
To: eculizumabfeedback

To Whom it May Concern;

I support PNH patients in New Zealand in their fight to gain access to the life saving treatment Soliris.

I DO NOT support PHARMAC's proposal to decline funding for the Soliris treatment.

I support the PNH Support Association's proposition for Equity and Fairness which states:

i. **PHARMAC must return to the negotiating table** with the supplier of the Soliris treatment

ii. **PHARMAC must negotiate in good faith** toward funding Soliris for a minimum of 8 NZ PNH patients

iii. **PHARMAC must establish fair assessment criteria**, based on expert advice from the international haematological community, to assess patient need for the Soliris treatment

iv. **PHARMAC must amend its Operating Policies and Procedures** to acknowledge the right of rare disease patients to access life restoring and life saving treatments as in the specific example of the Soliris treatment

Imagine Fighting for your child's life everyday, imagine spending every waking moment thinking of ways to fundraise enough money to keep your child alive each year and often sacrificing time with that child and/or your other children to do so and with absolutely no guarantees of success! The pain, I imagine would be massive!!

These drugs are funded in other counties so why not here when it's obvious that people are going to die without them.

Thanks for your time.

Yours faithfully

Kelly Richards

From: kerri richardson [redacted]
Sent: Tuesday, 4 June 2013 10:36 a.m.
To: eculizumabfeedback; OPP Review
Subject: PNH Support
Attachments: proposition_equity_and_fairness_-_email_submission_template.doc

Please email *BOTH*

Subject: Submission to PHARMAC's Proposal to decline a funding application for Soliris (eculizumab)
Email: eculizumabfeedback@pharmac.govt.nz

AND

Subject: Submission in response to PHARMAC's consultation on Decision Critera.
Email: opp@pharmac.govt.nz

To Whom it May Concern;

I support PNH patients in New Zealand in their fight to gain access to the life saving treatment Soliris.

I DO NOT support PHARMAC's proposal to decline funding for the Soliris treatment.

I support the PNH Support Association's proposition for Equity and Fairness which states:

i. **PHARMAC must return to the negotiating table** with the supplier of the Soliris treatment

ii. **PHARMAC must negotiate in good faith** toward funding Soliris for a minimum of 8 NZ PNH patients

iii. **PHARMAC must establish fair assessment criteria**, based on expert advice from the international haematological community, to assess patient need for the Soliris treatment

iv. **PHARMAC must amend its Operating Policies and Procedures** to acknowledge the right of rare disease patients to access life restoring and life saving treatments as in the specific example of the Soliris treatment

Yours faithfully

Kerri Richardson

From: Kim Karaitiana [redacted]
Sent: Sunday, 2 June 2013 9:09 p.m.
To: eculizumabfeedback
Subject: Re Pharmac
Attachments: .~lock.proposition_equity_and_fairness_-_email_submission_template.doc#

```
,KIMMIE/Nick,Kimmie,02.06.2013
21:02,file:///C:/Documents%20and%20Settings/Nick/Application%20Data/OpenOff
ice.org/3;
```

From: Kirsty Armstrong [redacted]
Sent: Thursday, 6 June 2013 12:00 p.m.
To: eculizumabfeedback
Subject: Funding of Soliris

To Whom it May Concern;

I support PNH patients in New Zealand in their fight to gain access to the life saving treatment Soliris.

I DO NOT support PHARMAC's proposal to decline funding for the Soliris treatment.

I support the PNH Support Association's proposition for Equity and Fairness which states:

i. **PHARMAC must return to the negotiating table** with the supplier of the Soliris treatment

ii. **PHARMAC must negotiate in good faith** toward funding Soliris for a minimum of 8 NZ PNH patients

iii. **PHARMAC must establish fair assessment criteria**, based on expert advice from the international haematological community, to assess patient need for the Soliris treatment

iv. **PHARMAC must amend its Operating Policies and Procedures** to acknowledge the right of rare disease patients to access life restoring and life saving treatments as in the specific example of the Soliris treatment

It is promoted that PHARMAC's role is to get better value for medicines so that the best health outcomes can be achieved from public money spent on medicines.
The decision criteria for funding is supposed to include the availability and suitability of existing medicines, PHARMAC is well aware that Soliris has no alternative. So it is either funded or people are left to die.

It is horrific that people can be told by a Board working from decision criteria that their lives are not worth saving, maybe the decision of PHARMAC would be different if the disease was one which was relatable yet the few people with it are the unlucky ones who have to deal with it not the decision makers.

I have been to fund raisers for [redacted] a toddler with this and have seen the vast efforts of friends, family and the general public to try to pool together enough money but in the real world that is a huge target and funding of Soliris is what is required to save that little boy.

Your Faithfully

Kirsty Armstrong

From: Larissa Isted [redacted]
Sent: Thursday, 4 July 2013 10:26 a.m.
To: eculizumabfeedback; OPP Review
Subject: Submission re PHN

I came across this information when I heard some news via social media of a sufferer of Paroxysmal Nocturnal Haemoglobinuria (PNH). I have not heard about this disease and wanted to do some further reading on this. I also thought it was also worthwhile to have a read through the site and PHARMAC's stance on this case. After reading the PHARMAC site and that of the PNH Support Association, I am happy to provide the following submission.

I support PNH patients in New Zealand in their fight to gain access to the life saving treatment Soliris.

I DO NOT support PHARMAC's proposal to decline funding for the Soliris treatment.

I support the PNH Support Association's proposition for Equity and Fairness which states:

i. **PHARMAC must return to the negotiating table with the supplier of the Soliris treatment**

ii. **PHARMAC must negotiate in good faith toward funding Soliris for a minimum of 8 NZ PNH patients**

iii. **PHARMAC must establish fair assessment criteria, based on expert advice from the international haematological community, to assess patient need for the Soliris treatment**

iv. **PHARMAC must amend its Operating Policies and Procedures to acknowledge the right of rare disease patients to access life restoring and lifesaving treatments as in the specific example of the Soliris treatment**

I am saddened to hear when a treatment that is available to help the lives of these people cannot be funded? It appears other countries are doing this, the number of cases are low in NZ and the success rate of the treatment to allow these people reasonable quality of life appears sound. After a complete and fair analysis is done if it cannot be fully funded what about part-funding to at least allow these individuals and their families a chance to life?

Every measure should be taken to give these people a fighting chance, PHARMAC should not abandon talks with a supplier of a lifesaving treatment.

Even though I do not know any of these sufferers personally, I hope to share this story with as many of my networks as I can.

Kind Regards
Larissa

Larissa Isted [redacted]
[redacted]

Please consider the environment before printing this e-mail

From: Laurel McDonald [redacted]
Sent: Monday, 24 June 2013 9:58 p.m.
To: eculizumabfeedback
Subject: Submission to PHARMAC's Proposal to decline a funding application for Soliris (eculizumab)

To Whom it May Concern;

I support PNH patients in New Zealand in their fight to gain access to the life saving treatment Soliris.

I DO NOT support PHARMAC's proposal to decline funding for the Soliris treatment.

I support the PNH Support Association's proposition for Equity and Fairness which states:

i. **PHARMAC must return to the negotiating table** with the supplier of the Soliris treatment

ii. **PHARMAC must negotiate in good faith** toward funding Soliris for a minimum of 8 NZ PNH patients

iii. **PHARMAC must establish fair assessment criteria**, based on expert advice from the international haematological community, to assess patient need for the Soliris treatment

iv. **PHARMAC must amend its Operating Policies and Procedures** to acknowledge the right of rare disease patients to access life restoring and life saving treatments as in the specific example of the Soliris treatment

There is a small but significant number of New Zealanders suffering from this disease who could improve the quality and quantity of life if they are able to gain access. I just hope and pray that money is not the only thing standing in the way of these people in need being able to get this drug they so desperately need. What is the cost of a life? Please seriously consider these matters. It is much appreciated.

Yours faithfully,

Laurel McDonald

From: Laura Lopes [redacted]
Sent: Tuesday, 30 July 2013 8:56 p.m.
To: eculizumabfeedback
Subject: Submission to PHARMAC'S proposal to decline a funding application for Soliris (eculizumab)

To whom it may concern:

The PNH patients in New Zealand have my full support in their fight to gain access to the lifesaving treatment Soliris.

I **DO NOT** support Pharmac's proposal to decline this treatment

- The statistics Pharmac quoted when they took this consultation to the public was misleading as it overstated the number of people affected with PNH which in turn over exaggerates the likely real cost of treatment. You did this back in 2011 when Pharmac staff reported to the Board that there would be up to 100 affected patients in New Zealand with Pompe disease. That treatment was declined, and it looks as though you intend to decline Soliris, again with dubious data as the basis for a decision. This is not acceptable and you should withdraw this consultation because of the misleading information in it, which is likely to skew responses from the public. I should be able to rely on Pharmac to provide accurate and reliable information that will help guide responses from the community.

- These patients, as all patients do, have a right to life. Our health system has a duty to address their needs in a fair and equitable way. When Pharmac places so much emphasis on costs, cost-effectiveness and alternative use of the money, but does not address issues of rights, equity, fairness, humanity and community values, you are failing in your duty. The District Health Boards have this duty, and Pharmac is acting as their purchasing agency. You should use the same decision criteria and priorities that they have.

- Patients with rare diseases are disadvantaged because their condition is rare, and when treatments become available they are doubly affected by the high cost and the very small market due to small numbers. It is a denial of their right to health, and contrary to goals of "Equity of access, reducing inequalities and improving health outcomes for individuals and communities will guide our relationship and decision making", which are set out in the agreement between DHBs and Pharmac about how you decide things on their behalf. They did not decide to be affected by their disease and in no way should be disadvantaged by it in regards to accessing treatment.

- **Decision Criteria:** The decision criteria used to assess medicines are not fair for those who are affected by rare diseases. There should be an additional layer of decision-making for rare diseases that do not fit the standard cost effectiveness threshold for large populations. These additional layers already exist in Australia, England, Scotland and other places around the world, because they have recognised that it's a fair way to deal with the disadvantage of rare diseases.
- Pharmac's assumption that "best health outcomes" as mentioned in its legislation, can be strongly associated with calculation of Quality Adjusted Life Years, is too narrow a view of what is "best", especially when Pharmac's calculations do not take into account non-health-sector costs. The decision criteria should include a broader range of considerations that are important to patients, so that the decisions are made in a patient-centred way.

- Pharmac also has a responsibility for "health outcomes that are reasonably achievable". Your narrow perspective on technical assessment and budget management, to the exclusion of patient rights and interests from decisions, are an outcome that effectively discriminates against patients with rare diseases, and is not a reasonable outcome by any measure.

- Pharmac often emphasises the "tough decision" approach that some will be funded and others not. That is too simplistic and can also be unfair, as it is in this case. There are very good reasons to adopt an equitable approach that spreads medicine funding across as many areas of health need as possible so that no group is completely abandoned. That is the approach across a wide range of services in our health system and Pharmac should adopt a similar approach, rather than exclude and abandon certain groups.

- **I do not support** your intent to decline treatment for this group or other patients where there are therapies for rare diseases.

Kind Regards,

.aura Lopes

[redacted]

From: Laura Stewart [redacted]
Sent: Friday, 31 May 2013 9:20 p.m.
To: eculizumabfeedback
Subject: Submission to PHARMAC's Proposal to decline a funding application for Soliris

To Whom it May Concern;
I support PNH patients in New Zealand in their fight to gain access to the life saving treatment Soliris.
I DO NOT support PHARMAC's proposal to decline funding for the Soliris treatment.
I support the PNH Support Association's proposition for Equity and Fairness which states:
PHARMAC must return to the negotiating table with the supplier of the Soliris treatment

PHARMAC must negotiate in good faith toward funding Soliris for a minimum of 8 NZ PNH patients

PHARMAC must establish fair assessment criteria, based on expert advice from the international haematological community, to assess patient need for the Soliris treatment

PHARMAC must amend its Operating Policies and Procedures to acknowledge the right of rare disease patients to access life restoring and life saving treatments as in the specific example of the Soliris treatment

Yours faithfully

Laura Webster

From: [redacted]
Sent: Wednesday, 12 June 2013 8:49 a.m.
To: eculizumabfeedback
Subject: Soliris funding
Attachments: ATT00001.txt; ATT00002.htm

To Whom it May Concern;

I support PNH patients in New Zealand in their fight to gain access to the life saving treatment Soliris.

I DO NOT support PHARMAC's proposal to decline funding for the Soliris treatment.

I support the PNH Support Association's proposition for Equity and Fairness which states:

i. **PHARMAC must return to the negotiating table with the supplier of the Soliris treatment**

ii. **PHARMAC must negotiate in good faith toward funding Soliris for a minimum of 8 NZ PNH patients**

iii. **PHARMAC must establish fair assessment criteria, based on expert advice from the international haematological community, to assess patient need for the Soliris treatment**

iv. **PHARMAC must amend its Operating Policies and Procedures to acknowledge the right of rare disease patients to access life restoring and life saving treatments as in the specific example of the Soliris treatment**

As a parent of someone who lives with PNH I am saddened we have to continue to fight for the basic right to live. The cost of providing this drug to patients with PNH will have to be measured against the cost of not providing it. Without this drug patients need multiple blood transfusions and/or platelets. The cost of the chair they sit in while receiving the transfusions, the transfusions themselves, and the nursing staff involved in administering the transfusions, and the cost of missed time from work while these people are travelling to multiple blood work appointments is astounding. When these patients are sick they miss time from work thus placing more of a burden on the system with having jobs not done or having to find other people to cover their jobs. Their income is lower and the amount of taxes they pay is also decreased. Everyone benefits from healthy participants in the work force. I am confident you will see the benefit to providing Soliris to these people.

Yours faithfully

[redacted] [redacted]

Laura Zacharias, [redacted]
[redacted]

From: Lisa [redacted]
Sent: Tuesday, 30 July 2013 8:39 p.m.
To: eculizumabfeedback
Subject: Submission to Pharmac from LDNZ - July 2013.docx
Attachments: Submission to Pharmac from LDNZ - July 2013.docx; ATT00001.txt

To provide feedback, please submit it in writing by **Wednesday, 31 July 2013** to:

Sue Anne Yee
Therapeutic Group Manager
PHARMAC

Email: eculizumabfeedback@pharmac.govt.nz
Fax: 04 460 4995
Post: PO Box 10 254, Wellington 6143

Subject: **Submission to PHARAMC'S proposal to decline a funding application for Soliris (eculizumab)**

To whom it may concern:

I support the PNH patients in New Zealand in their fight to gain access to the lifesaving treatment Soliris.

I **DO NOT** support Pharmac's proposed intent to decline this treatment

- When Pharmac take this type of consultation to the public I should be able to rely on Pharmac to provide accurate and reliable information that will help guide responses from the community. In this consultation you have overstated the number of people affected with PNH which in turn over exaggerates the likely real cost of treatment. You did this back in 2011 when Pharmac staff reported to the Board that there would be up to 100 affected patients in New Zealand with Pompe disease. That treatment was declined, and it looks as though you intend to decline Soliris, again with dubious data as the basis for a decision. This is not acceptable and you should withdraw this consultation because of the misleading information in it, which is likely to skew responses from the public. .

- These patients have a right to life. Our health system has a duty to address their needs in a fair and equitable way. When Pharmac places so much emphasis on costs, cost-effectiveness and alternative use of the money, but does not address issues of rights, equity, fairness and community values, you are failing in your duty. The District Health Boards have this duty, and Pharmac is acting as their purchasing agency. You should use the same decision criteria and priorities that they have.

- Patients with rare diseases are disadvantaged because their condition is rare, and when treatments become available they are doubly affected by the high cost and the very small market due to small numbers. It is a denial of their right to health, and contrary to goals of "Equity of access, reducing inequalities and improving health outcomes for individuals and communities will guide our relationship and decision making", which are set out in the agreement between DHBs and Pharmac about how you decide things on their behalf.

- **Decision Criteria:** The decision criteria used to assess medicines are not fair for those who are affected by rare diseases. There should be an additional layer of decision-making for rare diseases that do not fit the standard cost effectiveness threshold for large populations. These additional layers already exist in Australia, England, Scotland and other places around the world, because they have recognised that it's a fair way to deal with the disadvantage of rare diseases.

- Pharmac's assumption that "best health outcomes" as mentioned in its legislation, can be strongly associated with calculation of Quality Adjusted Life Years, is too narrow a view of what is "best", especially when Pharmac's calculations do not take into account non-health-sector costs. The decision criteria should include a broader range of considerations that are important to patients, so that the decisions are made in a patient-centred way.

- Pharmac also has a responsibility for "health outcomes that are reasonably achievable". Your narrow perspective on technical assessment and budget management, to the exclusion of patient rights and interests from decisions, are an outcome that effectively discriminates against patients with rare diseases, and is not a reasonable outcome by any measure.

- Pharmac often emphasises the "tough decision" approach that some will be funded and others not. That is too simplistic and can also be unfair, as it is in this case. There are very good reasons to adopt an equitable approach that spreads medicine funding across as many areas of health need as possible so that no group is completely abandoned. That is the approach across a wide range of services in our health system and Pharmac should adopt a similar approach, rather than exclude and abandon certain groups.

- **I do not support** your intent to decline treatment for this group or other patients where there are therapies for rare diseases.

I strongly endorse this submission.

Warm regards
Lisa Martin

Sent from my iPad

From: Matthew & Leesa Collis [redacted]
Sent: Thursday, 20 June 2013 7:50 a.m.
To: eculizumabfeedback
Subject: Submission to PHARMAC's Proposal to decline a funding application for Soliris (eculizumab)

To Whom it May Concern;

I support PNH patients in New Zealand in their fight to gain access to the life saving treatment Soliris.

I DO NOT support PHARMAC's proposal to decline funding for the Soliris treatment.

I support the PNH Support Association's proposition for Equity and Fairness which states:

i. **PHARMAC must return to the negotiating table** with the supplier of the Soliris treatment

ii. **PHARMAC must negotiate in good faith** toward funding Soliris for a minimum of 8 NZ PNH patients

iii. **PHARMAC must establish fair assessment criteria**, based on expert advice from the international haematological community, to assess patient need for the Soliris treatment

iv. **PHARMAC must amend its Operating Policies and Procedures** to acknowledge the right of rare disease patients to access life restoring and life saving treatments as in the specific example of the Soliris treatment

Yours faithfully

Leesa Collis

From: Amy Munro [redacted]
Sent: Wednesday, 31 July 2013 2:38 p.m.
To: eculizumabfeedback
Cc: Pru Etcheverry
Subject: Submission regarding eculizumab proposal
Attachments: Submission from LBC re eculizumab.pdf

Good afternoon Sue Anne,

Please find attached submission from Leukaemia & Blood Cancer New Zealand regarding PHARMAC's proposal to decline funding for eculizumab for New Zealanders living with paroxysmal nocturnal haemoglobinurea (PNH).

Kind regards,
Amy

Amy Munro
Support Services Manager

Leukaemia & Blood Cancer New Zealand
6 Claude Road, Epsom, Auckland 1023 | PO Box 99182, Newmarket, Auckland 1149 | leukaemia.org.nz | shaveforacure.co.nz |
[redacted]

[redacted] [redacted] [redacted] [redacted]

LBC thanks Bell Gully, Farmers, Fidelity Life, .99, PricewaterhouseCoopers, SKYCITY, Suzuki & The Textile Recycling Centre for their valued support.

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leukaemia &
blood cancer
new zealand
our mission is to care, our vision is to cure

National Office
[redacted]

31 July 2013

Sue Anne Yee
Therapeutic Group Manager
PHARMAC
PO Box 10254
WELLINGTON 6143
By email: eculizumabfeedback@pharmac.govt.nz

Dear Sue Anne,

Thank you for the opportunity to submit feedback regarding PHARMAC's proposal to decline a funding application for eculizumab (Soliris) for the treatment of paroxysmal nocturnal haemogloblinurea (PNH).

PNH is a very rare, severe and debilitating disease that until recently has had severely limited and suboptimal treatment options. Leukaemia & Blood Cancer New Zealand (LBC) is disappointed in the decision by PHARMAC to decline funding for eculizumab, and requests that this be reconsidered.

Eculizumab has clearly been shown in Phase III randomised controlled trials to reduce haemolysis, improve symptoms and quality of life, reduce the risk of thrombosis and reduce the number of red blood cell transfusions required. Eculizumab cannot be compared to other treatments as there is no comparator. Patients may now be offered a bone marrow transplant which is not the treatment of choice in this setting.

We acknowledge that eculizumab is an extremely expensive drug, and for this reason reason needs to be reviewed using different criteria to those applied to other pharmaceutical agents. In the first instance we urge PHARMAC to more carefully review the numbers of patients in New Zealand living with PNH who would benefit from treatment with eculizumab. We believe, following consultation with the New Zealand haematology community, that the 12-20 patients requiring eculizumab mentioned in PHARMAC's proposal is an overestimation. Your document mentions overall prevalence of 60-70 patients which is very substantially beyond the patient numbers understood to be in New Zealand.

Of the 22 known patients who are listed on the New Zealand PNH national registry, it has been estimated that eight of these patients would be eligible for treatment with eculizumab if New Zealand were to adopt guidelines for treatment similar to those currently in place in Australia.

The Australian guidelines approve treatment with eculizumab for PNH using a number of criteria including PNH clone size; LDH value (reflecting cell breakdown rates); dependency on red cell transfusion; and whether or not the patient has had a major thrombotic episode.

Therefore, as there are a) significantly lower numbers of patients, and b) an overstated cost, the overall budgetary impact of treating these patients with eculizumab will likely be less than half of what has been suggested in PHARMAC's consultation document.

There are haematologists who are highly motivated to work with you to establish a set of criteria around the prescription of eculizumab for PNH patients in New Zealand, and we fully support this initiative.

It is acknowledged that the cost alone the of eculizumab, irrespective of the number of patients indicated, makes it very difficult to get across the line in terms of the funding assessment strategies used by PHARMAC. Other drugs for rare disorders are in a similar camp. PHARMAC needs different criteria for funding decisions around high cost, highly specialised medicines that benefit small numbers of patients, such as eculizumab.

Solutions need to be found for these types of drugs that are life-saving for patients, who through no fault of their own are diagnosed with rare and difficult to treat diseases. These New Zealanders don't deserve less of our energies and compassion in aiming to find treatment solutions for them.

We know that New Zealand can't afford everything that comes along. Leukaemia & Blood Cancer New Zealand only advocates for drugs that are highly efficacious in their setting and most particularly where there are no alternative treatments available. We believe each individual deserves the right to good health and it seems somewhat offensive to compare the well-being of a very small number of individuals with being able to provide healthcare to 40,000 others.

Eculizumab, which is funded in many other countries, including Australia, has wide medical benefits for patients. This includes fewer symptoms, prolonged survival, increased quality of life, and decreased costly hospital admissions. Please see over the page for comments from two New Zealanders whose lives would be changed forever with the funding of eculizumab.

Leukaemia & Blood Cancer New Zealand acknowledges and commends PHARMAC on its decision to seek public opinion on this very difficult issue. We sincerely hope this consultation process is genuine because once again we find ourselves having the same conversation with you about access to high cost, highly specialised medicines. We encourage PHARMAC to continue discussions with the drug sponsor on how eculizumab might be funded in New Zealand and to communicate actively with those fighting for access for this drug. We look forward to hearing PHARMAC's next steps in this funding decision.

Yours sincerely,

Pru Etcheverry

Pru Etcheverry
Chief Executive Officer

[redacted]

[redacted]

From: liam [redacted]
Sent: Saturday, 1 June 2013 7:28 p.m.
To: eculizumabfeedback
Subject: Submission to PHARMAC's Proposal to decline a funding application for Soliris (eculizumab)

To Whom it May Concern;

I support PNH patients in New Zealand in their fight to gain access to the life saving treatment Soliris.

I DO NOT support PHARMAC's proposal to decline funding for the Soliris treatment.

I support the PNH Support Association's proposition for Equity and Fairness which states:

i.
PHARMAC must return to the negotiating table with the supplier of the Soliris treatment

ii.
PHARMAC must negotiate in good faith toward funding Soliris for a minimum of 8 NZ PNH patients

iii.
PHARMAC must establish fair assessment criteria, based on expert advice from the international haematological community, to assess patient need for the Soliris treatment

iv.
PHARMAC must amend its Operating Policies and Procedures to acknowledge the right of rare disease patients to access life restoring and life saving treatments as in the specific example of the Soliris treatment

(please include your personal statements here)

Yours faithfully

Liam Mckeown

From: [redacted] [redacted]
Sent: Tuesday, 4 June 2013 8:14 a.m.
To: eculizumabfeedback
Subject: Submission to PHARMAC's Proposal to decline a funding application for Soliris (eculizumab)

To Whom it May Concern;

I support PNH patients in New Zealand in their fight to gain access to the life saving treatment Soliris.

I DO NOT support PHARMAC's proposal to decline funding for the Soliris treatment.

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iii. **PHARMAC must establish fair assessment criteria**, based on expert advice from the international haematological community, to assess patient need for the Soliris treatment

iv. **PHARMAC must amend its Operating Policies and Procedures** to acknowledge the right of rare disease patients to access life restoring and life saving treatments as in the specific example of the Soliris treatment

My niece suffers from PNH and is only in her [redacted] she's experienced much illness in her short life and needs to be given a chance at a better quality of life. [redacted] and her parents should not need the additional worry of their unwell daughter. Please, PLEASE reconsider and make this much needed treatment available.

I respectfully request that my email be noted and considered.

Yours faithfully

[redacted]
Gold Coast, Australia

From: lindsay brown [redacted]
Sent: Tuesday, 4 June 2013 6:28 a.m.
To: eculizumabfeedback
Subject: PNH patients

Hello,

This matter was brought to my attention in a class at the University of Nevada, Reno. I encourage that this medication be available to people who suffer from PNH. We need to save lives not destroy them. It is inhuman to withhold life saving medication.

Thank you,

Lindsay Brown
[redacted]

Sent from my iPhone

From: lisa archer [redacted]
Sent: Saturday, 27 July 2013 9:06 p.m.
To: eculizumabfeedback
Subject: Proposal to decline a funding application for eculizumab

I support the PNH patients in New Zealand in their fight to gain access to the lifesaving treatment eculizumab.

I <u>**DO NOT**</u> support Pharmac's proposed intent to decline this treatment.

- When Pharamc takes this type of consultation to the public I should be able to rely on Pharmac to provide accurate and reliable information that will help guide responses from the community. In this consultation you have overstated the number of people affected with PNH which in turn over exaggerates the likely real cost of treatment. You did this back in 2011 when Pharmac staff reported to the Board that there would be up to 100 affected patients in New Zealand with Pompe disease. That treatment was declined, and it looks as though you intend to decline eculizumab, again with dubious data as the basis for a decision. This is not acceptable and you should withdraw this consultation because of the misleading information in it, which is likely to skew responses from the public.
- These patients have a right to life. Our health system has a duty to address their needs in a fair and equitable way. When Pharmac places so much emphasis on costs, cost-effectiveness and alternative use of the money, but does not address issues of rights, equity, fairness and community values; you are failing in your duty. The District Health Boards have this duty, and Pharmac is acting as their purchasing agency. You should use the same decision criteria and priorities that they have. We give criminals the right to life if they get sick, we provide treatment. How, as a humane, developed country can we justify "Recommended that the application be declined because although it is an effective treatment, it is extremely expensive", you cannot put a price on saving/preserving a life. Try living with a child who has no options other than death because they have a rare disease. How dare you say it is too expensive to provide effective treatment. Cut the health care to criminals and provide treatment to those suffering unnecessarily, unjustifiably.

- Patients with rare diseases are disadvantaged because their condition is rare, and when treatments become available they are doubly affected by the high cost and the very small market due to small numbers. It is a denial of their right to health, and contrary to goals of "Equity of access, reducing inequalities and improving health outcomes for individuals and communities will guide our relationship and decision making", which are set out in the agreement between DHBs and Pharmac about how you decide things on their behalf.

- **Decision Criteria:** The decision criteria used to assess medicines are not fair for those who are affected by rare diseases. There should be an additional layer of decision-making for rare diseases that do not fit the standard cost effectiveness threshold for large populations. These additional layers already exist in Australia, England, Scotland and other places around the world, because they have recognised that it's a <u>fair way</u> to deal with the disadvantage of rare diseases.

- Pharmac's assumption that "best health outcomes" as mentioned in its legislation, can be strongly associated with calculation of Quality Adjusted Life Years, is too narrow a view of what is "best", especially when Pharmac's calculations do not take into account non-health-sector costs. The decision criteria should include a broader range of considerations that are important to patients, so that the decisions are made in a patient-centred way.
- Pharmac also has a responsibility for "health outcomes that are reasonably achievable". Your narrow perspective on technical assessment and budget management, to the exclusion of patient rights and interests from decisions, are an outcome that effectively discriminates against patients with rare diseases, and is not a reasonable outcome by any measure.
- Pharmac often emphasises the "tough decision" approach that some will be funded and others not. That is too simplistic and can also be unfair, as it is in this case. There are very good reasons to adopt an equitable approach that spreads medicine funding across as many areas of health need as possible, so that no group is completely abandoned. That is the approach across a wide range of services in our health system and Pharmac should adopt a similar approach, rather than exclude and abandon certain groups.
-

<u>I do not support your intent to decline treatment</u> for the PNH group or other patients where there are therapies for rare diseases.

Regards
Lisa Archer

From: Lisa Cross [redacted]
Sent: Saturday, 1 June 2013 8:05 a.m.
To: eculizumabfeedback
Subject: Feedback

To Whom it May Concern;

I support PNH patients in New Zealand in their fight to gain access to the life saving treatment Soliris.

I DO NOT support PHARMAC's proposal to decline funding for the Soliris treatment.

I support the PNH Support Association's proposition for Equity and Fairness which states:

i. **PHARMAC must return to the negotiating table with the supplier of the Soliris treatment**

ii. **PHARMAC must negotiate in good faith toward funding Soliris for a minimum of 8 NZ PNH patients**

iii. **PHARMAC must establish fair assessment criteria, based on expert advice from the international haematological community, to assess patient need for the Soliris treatment**

iv. **PHARMAC must amend its Operating Policies and Procedures to acknowledge the right of rare disease patients to access life restoring and life saving treatments as in the specific example of the Soliris treatment**

I find it absolutely abhorrent that people are told there life is not good enough to extend for another 30 years, especially when it has been shown that Soliris may not be needed for the long a term.

Yours faithfully

Lisa Cross

[redacted]

From: Lisa Macready [redacted]
Sent: Thursday, 6 June 2013 10:10 a.m.
To: eculizumabfeedback
Subject: Submission to PHARMAC's Proposal to decline a funding application for Soliris (eculizumab)

To Whom it May Concern;

I support PNH patients in New Zealand in their fight to gain access to the lifesaving treatment Soliris.

I DO NOT support PHARMAC's proposal to decline funding for the Soliris treatment.

I support the PNH Support Association's proposition for Equity and Fairness which states:

i. **PHARMAC must return to the negotiating table with the supplier of the Soliris treatment**

ii. **PHARMAC must negotiate in good faith toward funding Soliris for a minimum of 8 NZ PNH patients**

iii. **PHARMAC must establish fair assessment criteria, based on expert advice from the international haematological community, to assess patient need for the Soliris treatment**

iv. **PHARMAC must amend its Operating Policies and Procedures to acknowledge the right of rare disease patients to access life restoring and lifesaving treatments as in the specific example of the Soliris treatment**

Soliris can be used to save and improve the quality of many lives, including the life of a little boy named [redacted]. He is only [redacted]. Please help him to live long enough to vote for himself.

Yours faithfully

Lisa Macready
[redacted]

[redacted]

[redacted]

From: Lisa Marie Anderson [redacted]
Sent: Friday, 31 May 2013 9:29 p.m.
To: eculizumabfeedback
Subject: : Submission to PHARMAC's Proposal to decline a funding application for Soliris (eculizumab

To Whom it May Concern;

I support PNH patients in New Zealand in their fight to gain access to the life saving treatment Soliris.

I DO NOT support PHARMAC's proposal to decline funding for the Soliris treatment.

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iv. **PHARMAC must amend its Operating Policies and Procedures** to acknowledge the right of rare disease patients to access life restoring and life saving treatments as in the specific example of the Soliris treatment

I think it is disgusting and disgraceful to not give a small boy like [redacted] a fighting chance in life with this much needed drug. Every moment of everyday people are fighting for him to raise the money. The selfishness and greed is unfathomable and I absolutely live in hope for a positive outcome for PNH patients and their distraught families.

Yours faithfully

Lisa Marie Anderson.

From: [redacted]
Sent: Thursday, 20 June 2013 9:08 p.m.
To: eculizumabfeedback
Subject: Submission to Pharmac's proposal to decline a funding application for Soliris
Attachments: 20130620095821028.pdf

>

20 June 2013

To Whom It May Concern;

I support PNH patients in New Zealand in their fight to gain access to the life saving treatment Soliris.

I DO NOT support PHARMAC's proposal to decline funding for the Soliris treatment.

I support the PNH Support Association's proposition for Equity and Fairness which states:

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iv. PHARMAC must amend its Operating Policies and Procedures to acknowledge the right of rare disease patients to access life restoring and life saving treatments as in the specific example of the Soliris treatment

Yours faithfully

Lorna Grové

From: [redacted]
Sent: Wednesday, 3 July 2013 6:59 p.m.
To: eculizumabfeedback
Subject: funding

To Whom it May Concern
The availability of the drug eculizumab to those suffering from PNH is vital. My daughter aged [redacted] has PNH and was diagnosed about [redacted] years ago now. She is an Australian citizen and therefore has been fortunate to receive this life saving drug through the Australian Life Saving Drug program not without a long battle to get it passed through government. Without this drug she suffered severely and the difference is remarkable since going on Eculizumab her life expectancy has gone from 5 years to a long long life now.
I plead with you to reconsider your decision in New Zealand to not release this drug to the PNH sufferers in New Zealand –**peoples lives are at stake here** . There should be no question, yes it is expensive but you cannot put a price on someones life it is just unthinkable that this would be the case.

Regards
[redacted]

From: Jenny Noble [redacted]
Sent: Friday, 19 July 2013 10:15 a.m.
To: eculizumabfeedback
Subject: Submission to PHARAMC'S proposal to decline a funding application for Soliris (eculizumab)

To whom it may concern:

I support the PNH patients in New Zealand in their fight to gain access to the lifesaving treatment Soliris.

I <u>DO NOT</u> support Pharmac's proposed intent to decline this treatment.

- When Pharamc takes this type of consultation to the public I should be able to rely on Pharmac to provide accurate and reliable information that will help guide responses from the community. In this consultation you have overstated the number of people affected with PNH which in turn over exaggerates the likely real cost of treatment. You did this back in 2011 when Pharmac staff reported to the Board that there would be up to 100 affected patients in New Zealand with Pompe disease. That treatment was declined, and it looks as though you intend to decline Soliris, again with dubious data as the basis for a decision. This is not acceptable and you should withdraw this consultation because of the misleading information in it, which is likely to skew responses from the public.
- These patients have a right to life. Our health system has a duty to address their needs in a fair and equitable way. When Pharmac places so much emphasis on costs, cost-effectiveness and alternative use of the money, but does not address issues of rights, equity, fairness and community values; you are failing in your duty. The District Health Boards have this duty, and Pharmac is acting as their purchasing agency. You should use the same decision criteria and priorities that they have.

- Patients with rare diseases are disadvantaged because their condition is rare, and when treatments become available they are doubly affected by the high cost and the very small market due to small numbers. It is a denial of their right to health, and contrary to goals of "Equity of access, reducing inequalities and improving health outcomes for individuals and communities will guide our relationship and decision making", which are set out in the agreement between DHBs and Pharmac about how you decide things on their behalf.

- **Decision Criteria:** The decision criteria used to assess medicines are not fair for those who are affected by rare diseases. There should be an additional layer of decision-making for rare diseases that do not fit the standard cost effectiveness threshold for large populations. These additional layers already exist in Australia, England, Scotland and other places around the world, because they have recognised that it's a <u>fair way</u> to deal with the disadvantage of rare diseases.

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- Pharmac also has a responsibility for "health outcomes that are reasonably achievable". Your narrow perspective on technical assessment and budget management, to the exclusion of patient rights and interests from decisions, are an outcome that effectively discriminates against patients with rare diseases, and is not a reasonable outcome by any measure.

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<u>I do not support your intent to decline treatment</u> for the PNH group or other patients where there are therapies for rare diseases.

Jenny

Jenny Noble
Field Officer | Administrator
Lysosomal Diseases New Zealand
167 Hollister Lane
Ohauiti, Tauranga, New Zealand
Phone +64 [redacted]
Cell: +[redacted]
e-mail [redacted]
Website www.ldnz.org.nz

From: Marg & Barry [redacted]
Sent: Thursday, 18 July 2013 2:23 p.m.
To: eculizumabfeedback
Subject: Submission to PHARMAC's Proposal to decline a funding application for Soliris (eculizumab)

To Whom it May Concern;

I support PNH patients in New Zealand in their fight to gain access to the life saving treatment Soliris.

I DO NOT support PHARMAC's proposal to decline funding for the Soliris treatment.

I support the PNH Support Association's proposition for Equity and Fairness which states:

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PHARMAC must return to the negotiating table with the supplier of the Soliris treatment

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PHARMAC must negotiate in good faith toward funding Soliris for a minimum of 8 NZ PNH patients

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PHARMAC must establish fair assessment criteria, based on expert advice from the international haematological community, to assess patient need for the Soliris treatment

iv.
PHARMAC must amend its Operating Policies and Procedures to acknowledge the right of rare disease patients to access life restoring and life saving treatments as in the specific example of the Soliris treatment

I have a friend with PNH, and it is very difficult to see her in such constant pain. It is difficult to see such a positive happy bubbly person, and her family, going through hell.

It is difficult to believe that in this era, in a first world country, that life saving medication is denied to patients. And because of this, these patients lives have lower quality of life, and die.

All New Zealanders have a right to fair and equitable access to healthcare.

Please reconsider, think of the patients and their families. These are real people, not just numbers and budgets.

Yours faithfully

Margaret Francis

Sent from my iPad

From: [redacted]
Sent: Thursday, 20 June 2013 5:05 a.m.
To: eculizumabfeedback
Subject: proposal to decline funding for Soliris
Attachments: submission (final copy).docx

To whom it may concern:

I am [redacted] the mother of [redacted] who suffers from the acquired blood disease PNH.

What can anyone say by way of a submission when their first born child's life is at stake?

What can I say to you that would change your mind, and fund this drug Soliris for my daughter and her fellow sufferers?

All I can say is I have seen her suffer.

I have seen her suffer as a young adult when finally she was diagnosed, she never complained, or raged against the fairness of it all. She just was thankful, that she was finally diagnosed with the dreadful label we now know as PNH.

[redacted]

How, she has run her professional life and continued with its day-to-day demands, has been to me, an overwhelming display of her tenacity. What courage she has shown her family and friends. What perseverance. Her life thus far has been a triumph over adversity.

Surely, these are the values we promote to the young children of our society to work towards.

Now we are saying to her. Forget how much you have struggled and suffered because we, your government are not going to value your life thus far, or your efforts to endure, no matter what. We are not going to support you at all.

[redacted] However the most important job I could ever have in my life, that is being a mother and grandmother, has been potentially removed from me. Firstly by a disease that can be mitigated with drug treatment, and then by a Government funding group, who have other priorities.

All people are precious to someone. Your own children more so. My precious daughter is left to languish in a country that is not her homeland. Knowing she can never come home, as her Government will not support her in her medical needs. Knowing her family will always be out of her reach. Knowing her country has discarded her.

[redacted]

What stress for her. If she could give me her disease I would gladly take it. As this will never be an option, I ask you to look at my daughter and the PNH sufferers as people who need to be viewed as members of our society who are a yardstick of how we treat the most vulnerable members of our society. Grant them a reprieve and give them some support by funding the drug they desperately need to stay alive and loved.

To Whom it May Concern,

I support PNH patients in New Zealand in their fight to gain access to the life saving treatment soliris.

I DO NOT support PHARMAC's proposal to decline funding for the Soliris treatment.

I support the PNH Support Association's proposition for Equity and Fairness which states:

i) PHARMAC must return to the negotiating table with the supplier of the Soliris treatment
ii) PHARMAC must negotiate in good faith toward funding Soliris for a minimum of 8 NZ PNH patients
iii) PHARMAC must establish fair assessment criteria, based on expert advice from the international haematological community, to assess patient need for the Soliris treatment
iv) PHARMAC must amend its Operating Policies and Procedures to acknowledge the right of rare disease patients to access life restoring and life saving treatments as in the specific example of the Soliris treatment

Yours faithfully

[redacted]

From: Marianne Hannagan [redacted]
Sent: Tuesday, 30 July 2013 9:58 a.m.
To: eculizumabfeedback
Subject: Submission to Pharmac's proprosal to decline Soliris (Eculizumab)
Attachments: Soliris submission.docx

Morning Sue

Please find attached a submission for the above. Thank you.

Regards
Marianne Hannagan

RE: **SUBMISSION TO PHARMAC'S PROPOSAL TO DECLINE A FUNDING APPLICATION FOR SOLIRIS (ECULIZUMAB)**

I support PNH patients in New Zealand in their fight to gain access to the life saving treatment Soliris.

I **DO NOT** support PHARMAC's proposal to decline funding for the Soliris treatment.

I find it abhorrent that Pharmac should consider declining the funding of a medicine now or in the future on the basis that "we (Pharmac) understand that certainty is something that patients with PNH and their families would prefer". Uncertainty is a factor many patients within our health system continually confront.

PNH and other patient groups need transparency, equity and fairness in their quest for access to high cost medicines. These New Zealanders have had their lives and health outcomes significantly disadvantaged by long term chronic and very often severe health issues.

Those individuals with rare conditions who have been fortunate enough historically to have high cost medicines, (which do not meet the cost-benefit criteria) funded have sometimes been granted access to these pharmaceuticals on an ad hoc type basis, due to publicity and public outcry, with others following in their wake not being treated in a similar manner. This is not a helpful approach, as it does not address the underlying issues that make the system inadequate when dealing with expensive pharmaceuticals.

Pharmac needs a robust procedure applied to its decision making process when high cost medicines are involved, with transparency around outcomes and actual costs associated with those medicines. Expensive treatments are always going to prove extraordinarily difficult to provide an outcome everyone will be totally accepting of, but a blanket denial of access to those treatments for patients and families who desperately require these is not that solution, when it is based solely on cost. They are expensive medications developed predominantly for the benefit of few patients and are very often still under patent. Pharmac needs to do what it does best – **negotiate** with these pharmaceutical companies!

Other countries have begun treating these types of high cost pharmaceuticals differently from other mainstream medicines. New Zealand needs to look at the treatment of high cost medications in other countries. In Scotland, for example, they have established a new fund of £21 million to ensure that rare conditions have access to medicines and are not further disadvantaged due to the very high costs associated with these treatments (for year March 2013-April 2014).

No one would prefer to have an application to fund a medicine declined in order to deliver certainty. Seriously ill New Zealanders have their health and lives severely compromised and then have to live with the knowledge that there is a potentially life-saving drug, which is available in other countries, but not funded in New Zealand due to Pharmac's decision making criteria.

The cost-benefit ratio I agree is an important motive when assessing an individual's right to high cost health care. New Zealand has established itself as a country extremely capable of negotiating with drug companies. This is why Pharmac has been incredibly successful at making significant savings in the provision of healthcare for medicines for the bulk of New Zealand's population. Negotiations with Pharmaceutical companies for high cost medicines will always be more difficult for both sides to arrive at a mutually agreed price point but New Zealand has already proven itself as a skilled negotiator. Pharmac needs to go back to the negotiating table for those needing Soliris.

It is time Pharmac urgently re-visited its decision-making criteria around rare conditions and high cost medicines. Soliris and those who so urgently need access to it do not have time on their side, nor do other patients where there are credible therapies available, but beyond reach in New Zealand.

I **DO NOT** support PHARMAC's proposal to decline funding for the Soliris treatment.

Thank you for your time and consideration of the above.

From: marilyn owen [redacted]
Sent: Thursday, 6 June 2013 12:34 p.m.
To: eculizumabfeedback
Subject: submission to Pharmac's proposal

To Whom it May Concern;

I support PNH patients in New Zealand in their fight to gain access to the life saving treatment Soliris.

I DO NOT support PHARMAC's proposal to decline funding for the Soliris treatment.

I support the PNH Support Association's proposition for Equity and Fairness which states:

i. **PHARMAC must return to the negotiating table** with the supplier of the Soliris treatment

ii. **PHARMAC must negotiate in good faith** toward funding Soliris for a minimum of 8 NZ PNH patients

iii. **PHARMAC must establish fair assessment criteria**, based on expert advice from the international haematological community, to assess patient need for the Soliris treatment

iv. **PHARMAC must amend its Operating Policies and Procedures** to acknowledge the right of rare disease patients to access life restoring and life saving treatments as in the specific example of the Soliris treatment

(please include your personal statements here)

Yours faithfully

Marilyn Owen

To Whom it May Concern;

I support PNH patients in New Zealand in their fight to gain access to the life saving treatment Soliris.

I DO NOT support PHARMAC's proposal to decline funding for the Soliris treatment.

I support the PNH Support Association's proposition for Equity and Fairness which states:

i. **PHARMAC must return to the negotiating table** with the supplier of the Soliris treatment

ii. **PHARMAC must negotiate in good faith** toward funding Soliris for a minimum of 8 NZ PNH patients

iii. **PHARMAC must establish fair assessment criteria**, based on expert advice from the international haematological community, to assess patient need for the Soliris treatment

iv. **PHARMAC must amend its Operating Policies and Procedures** to acknowledge the right of rare disease patients to access life restoring and life saving treatments as in the specific example of the Soliris treatment

(please include your personal statements here)

Yours faithfully

Marilyn Owen

From: Mark Kroening [redacted]
Sent: Wednesday, 26 June 2013 5:09 a.m.
To: eculizumabfeedback
Subject: Submission to PHARMAC's Proposal to decline a funding application for Soliris (eculizumab)

To Whom it May Concern;

I support PNH patients in New Zealand in their fight to gain access to the life saving treatment Soliris.

I DO NOT support PHARMAC's proposal to decline funding for the Soliris treatment.

I support the PNH Support Association's proposition for Equity and Fairness which states:

i. **PHARMAC must return to the negotiating table** with the supplier of the Soliris treatment

ii. **PHARMAC must negotiate in good faith** toward funding Soliris for a minimum of 8 NZ PNH patients

iii. **PHARMAC must establish fair assessment criteria**, based on expert advice from the international haematological community, to assess patient need for the Soliris treatment

iv. **PHARMAC must amend its Operating Policies and Procedures** to acknowledge the right of rare disease patients to access life restoring and life saving treatments as in the specific example of the Soliris treatment

Yours faithfully

Mark Kroening

From: [redacted] on behalf of Mary Carter [redacted]
Sent: Tuesday, 30 July 2013 10:20 p.m.
To: eculizumabfeedback
Subject: Funding

Dear Sue,

I'm not too sure exactly how many people need this drug in New Zealand, but I am conflicted because I am aware of the stance and positioning you may be coming from and am also aware of where families who are in need of this drug are coming from.

I choose to support a child who needs this drug out of my own pocket every week and am saddened that in this day and age there is a child who's life would be beneficial (and perhaps give them a longer, stable life) because of Eculizumab. But why is it is not funded in our country?

I am aware that this is the single most expensive drug in the word at just over $500,000 and that there other drugs would be beneficial to support many people for this price. Through some research I can see that there are many people who this drug would benefit and I believe that their lives are worth making better or potentially give them a long and stable life.

To think that some of these people were working and paying taxes, then when push comes to shove, are in need of medical treatment, they are then told that their taxpaying dollars go to support other people and not them, that is a big blow. I feel that these people deserve a happy and healthy life and if that's what Eculizumab does for them, who are we to stop that.

I really hate the economy for forcing Alexico to have these sorts of prices. Who puts a price on someones wellbeing? Pharmac would be just as bad for not paying out for those families who need it although it costs alot. Where does it stop? Do you think that in 10 years you may decide to fund it, then these families have had 10 years (5million dollars per person) of unnecessary heartache and potential deaths because it couldn't be done sooner.

I hope that you review your proposal to decline. What about part subsidising it, it may be more manageable for families to find the money necessary. Imagine if you needed this drug for a family member but realised you declined it and then struggled to get people's support or enough money to fund it. Don't do this to them, they are struggling already.

Kind Regards,
Mary Carter

From: Kennard Matt [redacted]
Sent: Tuesday, 11 June 2013 8:59 p.m.
To: eculizumabfeedback
Subject: Submission to PHARMAC's Proposal to decline a funding application for Soliris (eculizumab)

To Whom it May Concern;

I support PNH patients in New Zealand in their fight to gain access to the life saving treatment Soliris.

I DO NOT support PHARMAC's proposal to decline funding for the Soliris treatment.

I support the PNH Support Association's proposition for Equity and Fairness which states:

i. **PHARMAC must return to the negotiating table** with the supplier of the Soliris treatment

ii. **PHARMAC must negotiate in good faith** toward funding Soliris for a minimum of 8 NZ PNH patients

iii. **PHARMAC must establish fair assessment criteria**, based on expert advice from the international haematological community, to assess patient need for the Soliris treatment

iv. **PHARMAC must amend its Operating Policies and Procedures** to acknowledge the right of rare disease patients to access life restoring and life saving treatments as in the specific example of the Soliris treatment

Please take this opportunity to make a difference.

Yours faithfully

Matt Kennard

From: Matt Scarr [redacted]
Sent: Saturday, 1 June 2013 2:31 p.m.
To: eculizumabfeedback
Subject: Submission to PHARMAC's Proposal to decline a funding application for Soliris (eculizumab)

To Whom it May Concern;

I support PNH patients in New Zealand in their fight to gain access to the life saving treatment Soliris.

I DO NOT support PHARMAC's proposal to decline funding for the Soliris treatment.

I support the PNH Support Association's proposition for Equity and Fairness which states:

i. **PHARMAC must return to the negotiating table** with the supplier of the Soliris treatment

ii. **PHARMAC must negotiate in good faith** toward funding Soliris for a minimum of 8 NZ PNH patients

iii. **PHARMAC must establish fair assessment criteria**, based on expert advice from the international haematological community, to assess patient need for the Soliris treatment

iv. **PHARMAC must amend its Operating Policies and Procedures** to acknowledge the right of rare disease patients to access life restoring and life saving treatments as in the specific example of the Soliris treatment

I understand the financial costs may be high, but I hate to think that as a society we would ever agree that money should be valued more highly than somebody's quality of life, let alone their life itself. Unfortunately many people, myself included, see this proposal as not only perpetuating this sentiment, but as a blatant and poorly considered sidestep away from the possibilities Soliris could offer these few, very unlucky people.

I follow the updates of a little boy, [redacted] (aka [redacted]), who suffers from [redacted] It is heartbreaking to know that his mother tries her best to scrape together funds for her boy, all the while being pushed back a step with news such as this proposal. I can only imagine how daunting and soul destroying it must be for her at times when a large organisation such as PHARMAC makes it clear that they consider her boy's life less important than the money that could be used to save him.

People suffering from such rare diseases only have *you* to count on. How empty and abandoned they must feel when their country, New Zealand of all places, denies them their right to survival, especially when treatment exists. At the very least they deserve a fair and *carefully* considered discussion between all relevant parties. Surely compassion still has a place in our lives.

Yours faithfully,

Matthew Scarr

From: Melvyn Bonner [redacted]
Sent: Monday, 8 July 2013 10:09 p.m.
To: eculizumabfeedback
Subject: Eculizumab Feedback

Dear Pharmac Board Members

Jan White, Anne Kolbe, Jens Mueller, Kura Denness, Stuart McLaughlan, David Kerr.

Are we now a 3rd world country where we can't provide medicine 41 other countries in the world can buy for their people.
Gee Pharmac that's a hard one to get around and explain really isn't it.
Not for Phamac it's not the explanation for it is quite simple the other 41 countries all gave into the lobbyists.
Really is that true or is it just possible the other countries have shown compassion for their people.
If Australia can afford to buy Soliris why can't we. Scaling up for population difference there are almost exactly the same percentage per population of PNH patients across the ditch. So they have the same cost model.
This is a national disgrace and has undermined my personal sense of security and well being and undermined my pride to be a New Zealander.
What about the little 11 year old girl in Auckland or the [redacted] your old dad in Auckland with [redacted].
I feel really sad when reading about that little 11 year old girl.
Why don't we have a high cost medicine policy?
Why don't you do your job and consider processes for improving access to high-cost, highly specialised medicines?
Why don't you listen to our own clinicians who support Soliris for our people?
Your mandate and vision needs an overhaul.
How about putting patients at the centre of the model instead of cost reduction.
How about putting a value on compassion and doing the right thing and supporting our population.
You know New Zealand had a proud history of leading the world in looking after its people but mow sadly under Pharmacs guidance we have slipped from world leader to below 41.

I do you hope this consultation process is genuine and not just a tick the box saying we've consulted so no we won't fund the medicine so bugger off.
This won't be going away any time soon our PNH patients support network is strong we are in for as long as it takes.
Not sure if you are replying to submissions but if you are please cover this off below in your responses.

Pharmac has written their own story on this and slanted all aspects to the view that enables them to evade responsibility to do the right thing.
Pharmac doesn't use compassion in the modelling.
Pharmac has overstated the cost of the medicine.
Pharmac has not taken into account in the cost modelling that the cost of the medicine is decreasing and at some point probably within 4 to 5 years it will be ultra cheap when the patent rolls over.
Pharmac has further hurt these 8 people by casting them as an unwanted burden on the health system
Pharmac has overstated the number of PNH patients in NZ there are 8 only with severe form of the disease.
Pharmac has understated the survival gain and a decade of research which points to an average survival gain of > 30 years.
Is our health system consigned to 3rd world Status from leading the world in healthcare to now be below 41st place.

Pharmac has stalled at every point with Soliris citing all sorts of stories like its doesn't work its unproven etc and when all those stories have been disproved by clinicians the cat is finally out of the bag a stroke of genius from Pharmac we are too poor to fund it we are below 41st in the world for health care.

Perhaps the board would like to go online at this link and read about Grace 11 in Auckland

http://www.pnhsanz.org.nz/the-faces-of-pnh1.html

Also have a look at what our Australian friends have said about the difference SolIris has made to their lives.

Regards
Mel Bonner

From: Jensen, Micah [redacted]
Sent: Wednesday, 5 June 2013 4:14 p.m.
To: eculizumabfeedback
Subject: Submission to PHARMAC's Proposal to decline a funding application for Soliris (eculizumab)

To Whom it May Concern;

I support PNH patients in New Zealand in their fight to gain access to the life saving treatment Soliris.

I DO NOT support PHARMAC's proposal to decline funding for the Soliris treatment.

I support the PNH Support Association's proposition for Equity and Fairness which states:

i. **PHARMAC must return to the negotiating table** with the supplier of the Soliris treatment

ii. **PHARMAC must negotiate in good faith** toward funding Soliris for a minimum of 8 NZ PNH patients

iii. **PHARMAC must establish fair assessment criteria,** based on expert advice from the international haematological community, to assess patient need for the Soliris treatment

iv. **PHARMAC must amend its Operating Policies and Procedures** to acknowledge the right of rare disease patients to access life restoring and life saving treatments as in the specific example of the Soliris treatment

Yours faithfully

Micah Jensen

Micah Jensen BSc BVSc MANZCVS (Avian Health)
Veterinary resident in avian, wildlife and zoo animal health
Institute of Veterinary, Animal & Biomedical Sciences
Phone 64[redacted]
Email [redacted]
Mail Private Bag 11-222, Palmerston North, New Zealand 4442
Website http://ivabs.massey.ac.nz | http://wildlife.massey.ac.nz

From: Michael McGurk [redacted]
Sent: Tuesday, 23 July 2013 1:55 p.m.
To: eculizumabfeedback
Subject: Submission to PHARMAC's Proposal to decline a funding application for Soliris (eculizumab)

To Whom it May Concern;

I support PNH patients in New Zealand in their fight to gain access to the life-saving treatment Soliris.

I DO NOT support PHARMAC's proposal to decline funding for the Soliris treatment.

I support the PNH Support Association's proposition for Equity and Fairness which states:

i. **PHARMAC must return to the negotiating table** with the supplier of the Soliris treatment

ii. **PHARMAC must negotiate in good faith** toward funding Soliris for a minimum of 8 NZ PNH patients

iii. **PHARMAC must establish fair assessment criteria,** based on expert advice from the international haematological community, to assess patient need for the Soliris treatment

iv. **PHARMAC must amend its Operating Policies and Procedures** to acknowledge the right of rare disease patients to access life restoring and life-saving treatments as in the specific example of the Soliris treatment

Yours faithfully

Michael McGurk,

Christchurch.

From: [redacted]
Sent: Wednesday, 19 June 2013 3:49 p.m.
To: eculizumabfeedback
Subject: Soliris for PNH Patients

I'm writing to let you know about my wife's story:
In [redacted] at age [redacted] (with [redacted] children under [redacted]) she was diagnosed with Aplastic Anaemia. She was desperately unwell at that time.
She made a slow recovery until 2003 when diagnosed with PNH.
Dependant on regular blood transfusions at that time she participated on a world wide trial of ecluzibab (soliris).
Ever since and receiving fortnightly treatments of Soliris she has lived an almost normal life. The change in her health was amazing!! Little or no symptoms now exist.
She is now [redacted] and we expect many more healthy years ahead. We regularly travel and are looking forward to watching our grandchildren etc.
We are indeed blessed that this treatment is available to her.
I hope you provide this life saving to your NZ patients as soon as possible.

[redacted]

BARRETT BAXTER BYE
CERTIFIED PRACTISING ACCOUNTANTS

8th Floor, 60 Albert Road, South Melbourne, Vic 3205
p: [redacted] | f: 03 9693 7733
e: [redacted] | www.barrettbaxterbye.com.au

From: Wilson, Michele [redacted]
Sent: Tuesday, 30 July 2013 2:14 p.m.
To: eculizumabfeedback
Cc: exec.director@nzord.org.nz
Subject: PNH
Attachments: Submission to Pharmac from LDNZ - July 2013.docx

To whom it may concern:

I do not support Pharmac's proposed intent to decline treatment to PNH patients. It is disappointing to think that companies such as Pharmac have the power to make a massive positive difference to the lives of these patients, but choose not to. I hope that you take a moment to imagine your own child, husband, wife, mother or father had this disease.. and reconsider.
Regards
Michele Wilson
PhD Student
Lincoln University

To provide feedback, please submit it in writing by **Wednesday, 31 July 2013** to:

Sue Anne Yee
Therapeutic Group Manager
PHARMAC

Email: eculizumabfeedback@pharmac.govt.nz
Fax: 04 460 4995
Post: PO Box 10 254, Wellington 6143

Subject: **Submission to PHARAMC'S proposal to decline a funding application for Soliris (eculizumab)**

To whom it may concern:

I support the PNH patients in New Zealand in their fight to gain access to the lifesaving treatment Soliris.

I **DO NOT** support Pharmac's proposed intent to decline this treatment

- When Pharmac take this type of consultation to the public I should be able to rely on Pharmac to provide accurate and reliable information that will help guide responses from the community. In this consultation you have overstated the number of people affected with PNH which in turn over exaggerates the likely real cost of treatment. You did this back in 2011 when Pharmac staff reported to the Board that there would be up to 100 affected patients in New Zealand with Pompe disease. That treatment was declined, and it looks as though you intend to decline Soliris, again with dubious data as the basis for a decision. This is not acceptable and you should withdraw this consultation because of the misleading information in it, which is likely to skew responses from the public. .

- These patients have a right to life. Our health system has a duty to address their needs in a fair and equitable way. When Pharmac places so much emphasis on costs, cost-effectiveness and alternative use of the money, but does not address issues of rights, equity, fairness and community values, you are failing in your duty. The District Health Boards have this duty, and Pharmac is acting as their purchasing agency. You should use the same decision criteria and priorities that they have.

- Patients with rare diseases are disadvantaged because their condition is rare, and when treatments become available they are doubly affected by the high cost and the very small market due to small numbers. It is a denial of their right to health, and contrary to goals of "Equity of access, reducing inequalities and improving health outcomes for individuals and communities will guide our relationship and decision making", which are set out in the agreement between DHBs and Pharmac about how you decide things on their behalf.

- **Decision Criteria:** The decision criteria used to assess medicines are not fair for those who are affected by rare diseases. There should be an additional layer of decision-making for rare diseases that do not fit the standard cost effectiveness threshold for large populations. These additional layers already exist in Australia, England, Scotland and other places around the world, because they have recognised that it's a fair way to deal with the disadvantage of rare diseases.

- Pharmac's assumption that "best health outcomes" as mentioned in its legislation, can be strongly associated with calculation of Quality Adjusted Life Years, is too narrow a view of what is "best", especially when Pharmac's calculations do not take into account non-health-sector costs. The decision criteria should include a broader range of considerations that are important to patients, so that the decisions are made in a patient-centred way.

- Pharmac also has a responsibility for "health outcomes that are reasonably achievable". Your narrow perspective on technical assessment and budget management, to the exclusion of patient rights and interests from decisions, are an outcome that effectively discriminates against patients with rare diseases, and is not a reasonable outcome by any measure.

- Pharmac often emphasises the "tough decision" approach that some will be funded and others not. That is too simplistic and can also be unfair, as it is in this case. There are very good reasons to adopt an equitable approach that spreads medicine funding across as many areas of health need as possible so that no group is completely abandoned. That is the approach across a wide range of services in our health system and Pharmac should adopt a similar approach, rather than exclude and abandon certain groups.

- **<u>I do not support</u>** your intent to decline treatment for this group or other patients where there are therapies for rare diseases.

From: Tom and Shell Kelly [redacted]
Sent: Thursday, 6 June 2013 2:16 p.m.
To: eculizumabfeedback
Subject: Submission to PHARMAC's Proposal to decline a funding application for Soliris (eculizumab)
Attachments: proposition_equity_and_fairness_-_email_submission_template.doc

To Whom it May Concern;

I support PNH patients in New Zealand in their fight to gain access to the life saving treatment Soliris.

I DO NOT support PHARMAC's proposal to decline funding for the Soliris treatment.

I support the PNH Support Association's proposition for Equity and Fairness which states:

i. **PHARMAC must return to the negotiating table** with the supplier of the Soliris treatment

ii. **PHARMAC must negotiate in good faith** toward funding Soliris for a minimum of 8 NZ PNH patients

iii. **PHARMAC must establish fair assessment criteria**, based on expert advice from the international haematological community, to assess patient need for the Soliris treatment

iv. **PHARMAC must amend its Operating Policies and Procedures** to acknowledge the right of rare disease patients to access life restoring and life saving treatments as in the specific example of the Soliris treatment

I cannot understand how PHARMAC can walk away from this. They must look beyond costs and budgets and realise that they have people's lives in their hands. Someone's, mother, father, son or daughter could be saved! I want to be able to say Im proud to be a New Zealander living in a country where our government can and will do what is in their power to help these people.

Yours faithfully

Michelle Kelly

[redacted]

From: Mike and Jo Grogan [redacted]
Sent: Monday, 29 July 2013 6:44 p.m.
To: OPP Review; eculizumabfeedback
Subject: Funding for rare diseases

Subject: Submission to PHARAMC'S proposal to decline a funding application for Soliris (eculizumab)
To whom it may concern:
I support the PNH patients in New Zealand in their fight to gain access to the lifesaving treatment Soliris.

I DO NOT support Pharmac's proposed intent to decl...ine this treatment. I also believe it would be a backward step for New Zealand as a whole.

New Zealand is a civilized democracy. We are part of the United Nations and pride ourselves on out human rights record. We hold our own on the world stage in every area of excellence in spite of being a small population. We have earned respect! Our public health system and our medicines purchasing agency (Pharmac), are held up as models by which other nations strive to aspire. However, on the issue of treatment for rare diseases, these other nations take the lead while we lethargically drag our heels. We are told by Pharmac that the issue is about cost and cost effectiveness, and there is no doubt that the new, innovative medicines are costly, not just to New Zealand, but to the international market. However, in spite of these high costs, other cash strapped nations appear to be meeting the challenge. This, in itself, makes our refusal to treat even more difficult to justify.

If we take the upholding of human rights seriously, we would agree that New Zealand citizens have a right to life. Hence, we have a moral obligation to ensure that everyone has access to life sustaining treatment when such treatments become available. No clinical population should ever be completely abandoned in our society. The notion that patients with rare diseases can be ignored because the costs are higher than average, is callous and inhumane. I recognize that it must be difficult at times to balance the books, but it would be morally wrong to achieve this by denying treatment to this small, select group of patients.

Patients with rare diseases are already disadvantaged because of deteriorating health and all that this entails; they should not be subjected to the added pressures of having to fight for the health benefits that most other New Zealanders take for granted.

As I see it, a far more compassionate approach would be for Pharmac to negotiate lower prices with drug companies, which incidentally is the precise expertise upon which Pharmac has built its international reputation. Come on Pharmac! Do the right thing! Take up this challenge and fund these drugs!

I do not support your intent to decline treatment for the PNH group or other patients where there are therapies for rare diseases.

Your sincerely

Jo Grogan

[redacted]

From: Miles Crawford [redacted]
Sent: Wednesday, 3 July 2013 11:45 a.m.
To: eculizumabfeedback; OPP Review
Subject: Fight to gain access to the life saving treatment Soliris

To Whom it May Concern;

I support PNH patients in New Zealand in their fight to gain access to the life saving treatment Soliris.

I DO NOT support PHARMAC's proposal to decline funding for the Soliris treatment.

I support the PNH Support Association's proposition for Equity and Fairness which states:

i. **PHARMAC must return to the negotiating table** with the supplier of the Soliris treatment

ii. **PHARMAC must negotiate in good faith** toward funding Soliris for a minimum of 8 NZ PNH patients

iii. **PHARMAC must establish fair assessment criteria**, based on expert advice from the international haematological community, to assess patient need for the Soliris treatment

iv. **PHARMAC must amend its Operating Policies and Procedures** to acknowledge the right of rare disease patients to access life restoring and life saving treatments as in the specific example of the Soliris treatment

Yours faithfully

Miles Crawford

From: Miriam Rodrigues [redacted] [redacted]
Sent: Wednesday, 31 July 2013 2:09 p.m.
To: eculizumabfeedback
Subject: Submission to PHARMAC's Proposal to decline a funding application for Soliris (eculizumab)

To Whom it May Concern;
I support PNH patients in New Zealand in their fight to gain access to the life saving treatment Soliris.
I DO NOT support PHARMAC's proposal to decline funding for the Soliris treatment.
I support the PNH Support Association's proposition for Equity and Fairness which states:
i. PHARMAC must return to the negotiating table with the supplier of the Soliris treatment
ii. PHARMAC must negotiate in good faith toward funding Soliris for a minimum of 8 NZ PNH patients
iii. PHARMAC must establish fair assessment criteria, based on expert advice from the international haematological community, to assess patient need for the Soliris treatment
iv. PHARMAC must amend its Operating Policies and Procedures to acknowledge the right of rare disease patients to access life restoring and life saving treatments as in the specific example of the Soliris treatment

regards,

Miriam Rodrigues

From: Dean Suhr [redacted]
Sent: Monday, 22 July 2013 6:29 a.m.
To: eculizumabfeedback; 0116444604995@efaxsend.com
Cc: NZORD John Forman; Jenny Noble
Subject: Comments on eculizumab
Attachments: New Zealand Pharmac - eculizumab (Soliris) 2013-07.pdf

Greetings Ms. Yee,

Attached please find our submission to the public comments on PHARMAC's proposed denial of access to eculizumab. We oppose this denial.

[see attached file: New Zealand Pharmac - eculizumab (Soliris) 2013-07.pdf]

Best Regards,

Dean

--
Dean Suhr
President
MLD Foundation
[redacted] [redacted]
[redacted]

All of the following can be found on our home page: http://MLDfoundation.org

- **Complete MLD information ...** testing, genetics, practical care ...
- **Now recruiting** ... ERT and 2 gene therapy clinical trials!
- Check out our **blog**
- Find links to our **Facebook**, **Pinterest** and more
- **Upcoming MLD Family Conferences™ & MLD Family Gatherings™**
    - United States – United States – August 2nd & 3rd in New Jersey. **REGISTER NOW!**
    - Europe – spring 2014

- **Donate While You Shop at Amazon** ... We earn a small referral fee if you use our link to start your Amazon order. We don't see any of the details and it costs you nothing. See the home page.

- **There are over 7,000 rare diseases ...** 50% of them affect children ... a third of those children won't live past the age of 5. Out of the 7,000 diseases only 400 of them have treatments. You can help make it 401, please donate on our home page.

We C.A.R.E. for MLD families around the globe ... for over 12 years!

MLD foundation

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West Linn, OR 97068-2878

+1 503-656-4808
FAX: +1 503-212-0159
deansuhr@MLDfoundation.org
www.MLDfoundation.org
A registered 501(c)(3) non-profit serving families worldwide

July, 21 2013

Sue Anne Yee
Therapeutic Group Manager
PHARMAC
PO Box 10 254, Wellington 6143
eculizumabfeedback@pharmac.govt.nz
Fax: 011 64 4 460 4995

Dear Ms. Yee,

The MLD Foundation supports and provides a voice to families around the world with MLD, a rare terminal neurometabolic lysosomal disease that most often affects infants at 18-24 months. Our tenants are encapsulated in our motto: We C.A.R.E. … Compassion for families, increasing Awareness, influencing & funding Research, and expanding Education for metachromatic leukodystrophy.

On behalf of the MLD patients and families in New Zealand **we are opposed to PHARMAC's proposed denial** of access to eculizumab (Soliris) for the treatment of paroxysmal nocturnal haemoglobinuria, another rare disease.

We are shocked to read PHARMAC's statement *"This proposal to decline the funding application is consistent with the clinical advice we have received, which recommended that the application be declined because although it is an effective treatment, it is extremely expensive."*

By your own analysis, the clinical conclusion is that **eculizumab "is an effective treatment."** PHARMAC has a responsibility for "health outcomes that are reasonably achievable" and the patients have a right to access effective therapies which your experts have determined to be the case with eculizumab.

PHARMAC's responsibility is to facilitate access to viable therapies in as cost effective manner as possible. Alexion, like every other rare disease therapy company, has numerous fiscal programs to assist those countries unable to provide fully for families that need access to their therapies. PHARMAC knows this yet relies on list prices to argue for denial. New Zealand's claim that they are a small country and cannot afford these relatively expensive therapies is offset by your smaller populations and resulting lower actual count of affected individuals. The prevalence of rare disease in New Zealand is no higher or lower than any other developed country so your fiscal challenges and access to tax funds scale exactly as they do here in the United States, Japan, Europe, or even Australia. Or is PHARMAC arguing that New Zealand arguing, based solely on population, that they are the fiscally the same as other similar populated countries under-developed countries like Central African Republic, Congo, Liberia. We think not. You, like every other developed country are wrestling with balancing budgets and setting priorities.

PHARMAC has clearly stated that the proposed denial is one based only on cost. Just as a stool does not stand on one leg, basing life saving and quality of life treatment decision on only one parameter does not make sense. Efficacy, accessibility to treatment, cost, quality of life, and impact on society of healthy productive individuals make a much more stable 5-legged stool. In fact, when one leg is more challenging, such as the cost issue in this case, the other four legs can provide stability and balance. PHARMAC's arguments about cost need to include total cost of not treating – both medical and loss of productivity to society, and they need to respect the rights of EVERY individual to be respected and equally cared for by society.

Why is an organization based in another country focused on another disease jumping in on this discussion? Two reasons. 1) We hosted a MLD Family Conference™ in New Zealand in 2011. We filled the meeting space at the Holiday Inn in Wellington with families of current and angel family members with MLD. This was the first time ever to have a national gathering of MLD families in New Zealand. Our meeting was very expensive to hold when travel expenses and time were considered, however, none of those families are less deserving than those we could get in our car and drive to. Supporting families with rare diseases requires these extraordinary efforts. 2) We, like PHARMAC and your District Health Boards, have an ethical and moral responsibility to, in a reasonable fashion, provide the services to families no matter where they live or their financial circumstances. That is why two of us, unpaid volunteers, literally traveled half way around the word to be with New Zealand families – and in adjacent years we went to Tokyo, Munich, and other cities outside the United States. And it is why we are reaching out today … every patient matters, no matter their disease. Less than 5% of the rare diseases have therapies – it is simply unacceptable that the handful of patients that can be treated might go without.

The number of patients with an individual rare disease is small and their individual voices may be soft, but rare disease patients have as much right to health services as those with chronic disease. In the United States the total cost of rare disease therapies, and we are charged the full list prices that New Zealand will most likely not be charged, is but a drop in the bucket compared to what is spent on cholesterol lowering drugs. Yes the prices are high, but the total cost impact is very low because the patient count is low. Taking drug prices out of context, and even extrapolating about patient prevalence must always be considered in context.

Further, please realize that the voice of the rare disease community is just beginning to be heard. Just as HIV/AIDS activists organized some 40 years ago, we are doing the same in the rare disease community. Worldwide, 1 in 10 people has one of the over 7,000 rare diseases. 1 in 10 is not something to be ignored, but consider that each of these patients has two parents, a neighbor, perhaps a teacher or a boss – that 4 or 5 in 10 that are directly impacted by rare disease. Dismissing this community disease by disease is not a good choice.

Further, you should feel fortunate that LDNZ – Lysosomal Disease New Zealand has such talented and committed individuals in their leadership. They literally travel the world to be informed about and influence research. This is not your typical "I read it on the Internet" organization. LDNZ is known, recognized, respected, active, connected, and contributing, not only on behalf of patients in New Zealand, but worldwide. You would be wise to heed their perspective and knowledge. We are in 100% full support of the detailed response LDNZ have provided to you with regard to the New Zealand specific issues surrounding this proposal. We cannot speak to the intimacies of the Kiwi health system and society, but we can add a global voice to the discussion.

We **DO NOT** support PHARMAC's proposed intent to decline access to eculizumab (Soliris) for the PNH group or other access for other rare disease patients where there are therapies.

Sincerely,

Dean Suhr, President
MLD Foundation

From: Monique Griffin [redacted]
Sent: Wednesday, 24 July 2013 8:47 a.m.
To: eculizumabfeedback
Subject: Reconsider saying yes to eculizumab

Dear PHARMAC:

I'm writing to you once again, to ask you to reconsider funding a treatment for a rare disease. Please reconsider funding eculizumab. I understand this treatment is expensive, but we all know the biotech firms are willing to work with you on cost. I also understand how well your business model for keeping costs downs has served your country in the past. However, that system is outdated and was never meant to handle rare diseases and their speciality medicines, because let's face it, you didn't take them into consideration when forming your well-oiled money saving machine. You didn't have to, they didn't exist. Times have changed and it is time for PHARMAC to figure out a way to provide their citizens with the medicines they need - speciality and non-speciality alike. The archaic model no longer works.

If specialized medicines do not fit your business model you need a new model. Sending patients home to die because they cannot be treated or cured with cheap aspirin or Viagra is inhumane in this day and age. Please reconsider funding this medicine along with all other speciality meds while you work on evolving your system. Why should New Zealanders have to suffer and die when there are effective treatments available. You need to find a way to fund this medicine, other countries have figured it out. I'm not saying it will be easy, cuts will need to be make to other areas, a lot of planning and reconfiguring will have to take place - but isn't allowing people to live their lives to the fullest worth that? Don't you want to be part of something meaningful that actually helps people rather than being the organization known as the government rationing board or the death panel?

You have an opportunity to actually positively impact peoples lives, to in essence save lives.

Thank you for your time.

Sincerely,
Monique Griffin

From: Monique Revell [redacted]
Sent: Thursday, 6 June 2013 9:16 a.m.
To: eculizumabfeedback
Subject: Submission to PHARMAC's Proposal to decline a funding application for Soliris (eculizumab)

To Whom it May Concern;
I support PNH patients in New Zealand in their fight to gain access to the life saving treatment Soliris.
I DO NOT support PHARMAC's proposal to decline funding for the Soliris treatment.
I support the PNH Support Association's proposition for Equity and Fairness which states:
PHARMAC must return to the negotiating table with the supplier of the Soliris treatment

PHARMAC must negotiate in good faith toward funding Soliris for a minimum of 8 NZ PNH patients

PHARMAC must establish fair assessment criteria, based on expert advice from the international haematological community, to assess patient need for the Soliris treatment

PHARMAC must amend its Operating Policies and Procedures to acknowledge the right of rare disease patients to access life restoring and life saving treatments as in the specific example of the Soliris treatment

I cannot understand why or how our govt can decline help for these few people that desperately need the help of Soliris treatment, If a parent refused to give their child health care that they desperately needed would they not be labeled with neglect and charged with manslaughter when their child passes away for not receiving critical treatment that they need? Then how can our govt get away with neglecting the rights of serious health treatment that is desperately needed and putting these peoples health at risk?!

Yours faithfully

Monique Revell

From: National Manager [redacted]
Sent: Wednesday, 31 July 2013 12:48 p.m.
To: eculizumabfeedback
Subject: Submission to Pharmac's proposal to decline funding for Soliris
Attachments: Pharmac letter Soliris.pdf

Importance: High

Nicole Millis
National Manager

Mucopolysaccharide &
Related Diseases Society
Aust. Ltd

PO Box 623
Hornsby NSW 1630
Australia
p.+61 [redacted]
f. +61 2 9476 8422
e. [redacted]
w. www.mpssociety.org.au

MPS AUSTRALIA

MPS
AUSTRALIA

Mucopolysaccharide
& Related Diseases Society
Aust. Ltd
Registered charity number
CFN 10916
ACN 064 723 146
ABN 76 064 723 146
Postal Address
PO Box 623 Hornsby
New South Wales 1630
Australia
Telephone 612 9476 8411
Facsimile 612 9476 8422

31 July 2013

Ms Sue Anne Yee
Therapeutic Group Manager
PHARMAC
Email: eculizumabfeedback@pharmac.govt.nz

To whom it may concern,

Re: SUBMISSION TO PHARMAC'S PROPOSAL TO DECLINE A FUNDING APPLICATION FOR SOLIRIS (ECULIZUMAB)

I represent the Mucopolysaccharide & Related Diseases Society of Australia. We are a non-profit organisation formed by patients, parents, relatives and friends of those affected by a range of rare, life-limiting genetic disorders known collectively as the mucopolysaccharide (or MPS) diseases.

As another rare disease patient group, the Society support the PNH patients in New Zealand in their fight to gain access to the lifesaving treatment Soliris.
The Society DOES NOT support Pharmac's proposed intent to decline this treatment.
We are also very concerned by reports from NZ rare disease patient groups, that Pharmac has a history of providing unreliable and misleading information, relating to patient numbers and cost of treatments (e.g. Pompe disease). Pharmac must be able to be relied upon to provide accurate and reliable information.

Across the world, patients with rare diseases are disadvantaged by the inherent nature of rare diseases. When treatments become available they are doubly affected by the high cost and the very small market due to small numbers. Yet clearly patients with rare disease have the same right to health and medical treatment (basic human rights) as anyone. It is also clear that a general assessment criteria for funding medicines is limited in its ability to cater for rare disease. Due to its inherent nature rare disease will never meet standard cost effectiveness requirements for large populations. This has already been acknowledged in many countries around the world including Australia which funds treatments for rare disease through its Life Saving Drugs Programme. PHARMAC must also find a way to more fairly provide funding for treatment for rare diseases in New Zealand.

The Society does not support your intent to decline lifesaving treatment for the PNH group or any other New Zealand patients where therapies exist for rare diseases.

Thank you for your consideration in this important matter.

Yours sincerely,

Nicole Millis
National Manager
e [redacted]
w www.mpssociety.org.au

A life is not measured by the number of days, months or years it is lived, but by the effect that life has on other people who touch it. It is not measured by the length of its days or the height of its achievements but by the breadth of its influence.

From: Chris Higgins [redacted]
Sent: Friday, 26 July 2013 2:04 p.m.
To: eculizumabfeedback
Subject: Submission to PHARMAC's Proposal to decline a funding application for Soliris (eculizumab)

To Whom it May Concern;

The Muscular Dystrophy Association of NZ (MDA NZ) supports PNH patients in New Zealand in their fight to gain access to the life saving treatment Soliris(eculizumab) .

MDA NZ DOES NOT support PHARMAC's proposal to decline funding for the Soliris treatment.

MDA NZ supports the PNH Support Association's proposition for Equity and Fairness which states:

i. **PHARMAC must return to the negotiating table** with the supplier of the Soliris treatment

ii. **PHARMAC must negotiate in good faith** toward funding Soliris for a minimum of 8 NZ PNH patients

iii. **PHARMAC must establish fair assessment criteria**, based on expert advice from the international haematological community, to assess patient need for the Soliris treatment

iv. **PHARMAC must amend its Operating Policies and Procedures** to acknowledge the right of rare disease patients to access life restoring and life saving treatments as in the specific example of the Soliris treatment

MDA NZ provides support for people with neuromuscular conditions, all of which are rare conditions that collectively affect approximately 4500 New Zealanders. MDA NZ is concerned that PHARMAC's processes and PHARMAC's response to the application for the funding of Soliris is indicative of PHARMAC's likely future approach for the funding of treatments for rare diseases. MDA NZ urges PHARMAC to consider and agree to the four points as outlined above by the PNH Support Association.

Yours Sincerely,

Chris Higgins
Chief Executive
Muscular Dystrophy Association of New Zealand Inc.
PO Box 12063, Penrose, AUCKLAND 1642
Phone: [redacted], Mobile: [redacted]
Free Ph: (0800) 800 337 www.mda.org.nz
Charities Commission Registration CC31123

MUSCULAR
DYSTROPHY
Association of New Zealand Inc
Te Ropu Mate Horokiwa o Aotearoa

Bow Tie 2013 let us know how it went!
Online survey: http://www.surveymonkey.com/s/JJ6GKH7

From: natalie williams [redacted]
Sent: Friday, 31 May 2013 9:35 p.m.
To: eculizumabfeedback
Subject: [redacted] is only [redacted] years old, his life can be saved.

To Whom it May Concern;

I support PNH patients in New Zealand in their fight to gain access to the life saving treatment Soliris.

I DO NOT support PHARMAC's proposal to decline funding for the Soliris treatment.

I support the PNH Support Association's proposition for Equity and Fairness which states:

i. **PHARMAC must return to the negotiating table** with the supplier of the Soliris treatment

ii. **PHARMAC must negotiate in good faith** toward funding Soliris for a minimum of 8 NZ PNH patients

iii. **PHARMAC must establish fair assessment criteria**, based on expert advice from the international haematological community, to assess patient need for the Soliris treatment

iv. **PHARMAC must amend its Operating Policies and Procedures** to acknowledge the right of rare disease patients to access life restoring and life saving treatments as in the specific example of the Soliris treatment

What would you do if you had a child with an illness that could be stopped by a drug that is available right now?! How would you feel if your child's life could be saved by this drug but you can't afford it and it won't be funded? How would you feel and what would you do? Would you give up? Please, please, you have the chance to do something wonderful here.

Yours faithfully

Natalie Anne Williams, [redacted].

From: Natasha Fernandes [redacted]
Sent: Friday, 31 May 2013 9:33 p.m.
To: eculizumabfeedback
Subject: Subject: Submission to PHARMAC's Proposal to decline a funding application for Soliris (eculizumab)

To Whom it May Concern;

I support PNH patients in New Zealand in their fight to gain access to the life saving treatment Soliris.

I DO NOT support PHARMAC's proposal to decline funding for the Soliris treatment.

I support the PNH Support Association's proposition for Equity and Fairness which states:

i. **PHARMAC must return to the negotiating table** with the supplier of the Soliris treatment

ii. **PHARMAC must negotiate in good faith** toward funding Soliris for a minimum of 8 NZ PNH patients

iii. **PHARMAC must establish fair assessment criteria**, based on expert advice from the international haematological community, to assess patient need for the Soliris treatment

iv. **PHARMAC must amend its Operating Policies and Procedures** to acknowledge the right of rare disease patients to access life restoring and life saving treatments as in the specific example of the Soliris treatment

Imagine if you guy had a loved one going through what these sufferers are going through. It's not fair that there is a medicine available for the PNH patients, but there's no support from the government or pharmaceutical companies to help fund or subsidize it. You have been blessed with good health, so pay it forward and fight for others who need your help. Please. A little fight from us will definitely go a long way for these families.

Yours faithfully

Natasha Fernandes

From: Natasha Gee [redacted]
Sent: Monday, 8 July 2013 1:55 p.m.
To: eculizumabfeedback; eculizumabfeedback
Subject: Soliris Treatment

To Whom it May Concern;

I support PNH patients in New Zealand in their fight to gain access to the life saving treatment Soliris.

I DO NOT support PHARMAC's proposal to decline funding for the Soliris treatment.

I support the PNH Support Association's proposition for Equity and Fairness which states:

i. **PHARMAC must return to the negotiating table** with the supplier of the Soliris treatment

ii. **PHARMAC must negotiate in good faith** toward funding Soliris for a minimum of 8 NZ PNH patients

iii. **PHARMAC must establish fair assessment criteria**, based on expert advice from the international haematological community, to assess patient need for the Soliris treatment

iv. **PHARMAC must amend its Operating Policies and Procedures** to acknowledge the right of rare disease patients to access life restoring and life saving treatments as in the specific example of the Soliris treatment

It is so unjust that there is a drug available to treat Paroxysmal Nocturnal Haemoglobinuria yet it is so highly priced that most patients have no access to this life saving medication.

Pharmac Board of Directors – what if it was your child/wife/husband/loved one that needed this treatment?

Yours faithfully

NATASHA GEE Managing Director
Skin Health Experts (S.H.E)
Office: (09)415 6757 Fax: (09)415 4373
DDI: [redacted]

S.H.E
SKIN HEALTH EXPERTS
dermalogica
eye slices

From: [redacted]
Sent: Saturday, 1 June 2013 9:25 p.m.
To: eculizumabfeedback
Subject: Submission to PHARMAC's Proposal to decline a funding application for Soliris (eculizumab)

To Whom it May Concern;

I support PNH patients in New Zealand in their fight to gain access to the life saving treatment Soliris.

I DO NOT support PHARMAC's proposal to decline funding for the Soliris treatment.

I support the PNH Support Association's proposition for Equity and Fairness which states:

i.
PHARMAC must return to the negotiating table with the supplier of the Soliris treatment

ii.
PHARMAC must negotiate in good faith toward funding Soliris for a minimum of 8 NZ PNH patients

iii.
PHARMAC must establish fair assessment criteria, based on expert advice from the international haematological community, to assess patient need for the Soliris treatment

iv.
PHARMAC must amend its Operating Policies and Procedures to acknowledge the right of rare disease patients to access life restoring and life saving treatments as in the specific example of the Soliris treatment

A [redacted] year old boy who is my good friends son is in desperate need of this treatment! He suffers every week and as a result is in and out of hospital! It is no life for an adult let lane a child! Why let New Zealanders suffer and die from a serious disease when it is not needed?! No one can afford Soliris! In result my friend has been trying to raise funding but at the end of the day, it's not going to cut it. There is no possible way for her to raise the amount needed every year. I urgently ask you to please reconsider the funding proposal

Yours faithfully
[redacted]

From: nathan brown [redacted]
Sent: Saturday, 22 June 2013 9:20 a.m.
To: eculizumabfeedback
Attachments: pnh.doc

Please email *BOTH*

Subject: Submission to PHARMAC's Proposal to decline a funding application for Soliris (eculizumab)
Email: eculizumabfeedback@pharmac.govt.nz

AND

Subject: Submission in response to PHARMAC's consultation on Decision Critera.
Email: opp@pharmac.govt.nz

To Whom it May Concern;

I support PNH patients in New Zealand in their fight to gain access to the life saving treatment Soliris.

I DO NOT support PHARMAC's proposal to decline funding for the Soliris treatment.

I support the PNH Support Association's proposition for Equity and Fairness which states:

i. **PHARMAC must return to the negotiating table** with the supplier of the Soliris treatment

ii. **PHARMAC must negotiate in good faith** toward funding Soliris for a minimum of 8 NZ PNH patients

iii. **PHARMAC must establish fair assessment criteria**, based on expert advice from the international haematological community, to assess patient need for the Soliris treatment

iv. **PHARMAC must amend its Operating Policies and Procedures** to acknowledge the right of rare disease patients to access life restoring and life saving treatments as in the specific example of the Soliris treatment

There are many humans in this world that do not deserve pity or our help, there are however those that are trying to live a good and healthy life. The fact that you seem to be able to place a monetary value on a human-being is nothing short of slave trading. (person x is worth more because they have good teeth while person b is worthless because he has bad teeth). I do not mean to sound dramatic but the funding of this drug will not only have a DIRECT impact on those afflicted with this disease but also those that they live and socialise with. What gives you the right to decide who lives and dies? There may only be eight cases in New Zealnd but there are also 8 people in New Zealand that need your help. I don't pretend to know it all but surely New Zealand has the heart and the economy to support these people.

Yours faithfully

Nathan Brown

From: Nathan [redacted]
Sent: Tuesday, 11 June 2013 5:42 p.m.
To: eculizumabfeedback
Subject: Submission to PHARMAC's Proposal to decline a funding application for Soliris (eculizumab)

To Whom it May Concern;

I support PNH patients in New Zealand in their fight to gain access to the life saving treatment Soliris.

I DO NOT support PHARMAC's proposal to decline funding for the Soliris treatment.

I support the PNH Support Association's proposition for Equity and Fairness which states:

i. **PHARMAC must return to the negotiating table** with the supplier of the Soliris treatment

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iii. **PHARMAC must establish fair assessment criteria**, based on expert advice from the international haematological community, to assess patient need for the Soliris treatment

iv. **PHARMAC must amend its Operating Policies and Procedures** to acknowledge the right of rare disease patients to access life restoring and life saving treatments as in the specific example of the Soliris treatment

Yours faithfully

Nathan G Spurdle

From: Ngaire Stirling [redacted]
Sent: Thursday, 11 July 2013 3:07 p.m.
To: eculizumabfeedback
Subject: please help

I wonder if these emails go anywhere but I assume a human being reads them. Hopefully someone with children not so jaded to believe that there isn't the possibility of funding happening for this life saving drug to keep this lovely little human alive. I write this for one boy, but I understand there are many that will benefit. [redacted]

Please help them.

Warm wishes
Ngaire Stirling
Editor & Owner of Brisbane Kids
[redacted]
http://www.brisbanekids.com.au

you can also sign up for regular updates here
[redacted]

Even when freshly washed and relieved of all obvious confections, children tend to be sticky. ~Fran Lebowitz

From: John Forman [redacted]
Sent: Monday, 29 July 2013 9:55 a.m.
To: Steffan Crausaz
Cc: Stuart McLauchlan; Sue Anne Yee; eculizumabfeedback
Subject: Your urgent attention required - Your consultation on a proposal to decline a funding application for eculizumab

Importance: High

The Chief Executive
Pharmac

Copies to Chair of the Pharmac board and the Eculizumab consultation team

Dear Steffan,

Your urgent attention required - Your consultation on a proposal to decline a funding application for eculizumab

You will be aware of issues NZORD has commented on publicly in a recent press release about errors and misleading information in the consultation document you sent out on 21 May 2013. NZORD was planning on commenting on these matters in our submission on the consultation document. However, the errors are so fundamental that NZORD has decided to formally draw these matters to your attention by way of this separate email.

Pharmac needs to ensure that people you consult with are adequately informed and NZORD considers revised information needs to be made available to submitters to ensure this is the case. We suggest you achieve this by withdrawing the consultation document, reissuing it and extending the consultation period. You could post this letter on your website to explain the extended consultation period. We think you should also draw the revised information to the attention of all of those to whom you sent the 21 May 2013 consultation document.

As you will know, one of the leading court cases on consultation in New Zealand is *Wellington International Airport v Air New Zealand*. That case set out useful statements on the standards for adequate consultation in New Zealand. One of the statements was:

> *Implicit in the concept [of consultation] is a requirement that the party consulted will be (or will be made) adequately informed so as to be able to make intelligent and useful responses. It is also implicit that the party obliged to consult, while quite entitled to have a working plan already in mind, must keep its mind open and be ready to change and even start afresh*

NZORD considers Pharmac needs to correct two fundamental errors in its consultation paper and provide additional information on the funding of eculizumab in other jurisdictions.

The first error is stating a likely treatment group of up to 20 patients. This conflicts with advice from your PTAC committee and their estimate of 3 patients per million, suggesting up to 13 patients in total. Your document exaggerates this by about 55%. The estimate of 13 patients is validated by a pro-rata population-based assessment of number actually treated in Australia, again concluding 13 patients in New Zealand.

The second error is stating the price of eculizumab at “more than $600,000 per patient per year” when it is known both to Pharmac and to NZORD that a confidential offer has been made for a discounted price to treat New Zealand patients. Though NZORD does not know the detail of this offer, it is clear that the cost is exaggerated in your document.

The combination of these two figures produces a cost of "approximately $12,000,000 (20 patients) per year" if eculizumab was funded. This cost estimate is a misleading statement. The calculation provides a figure for the treatment of patients that we understand overstates the costs (if you decided to fund treatment) by more than 100%.

Further we consider the statements from PTAC minutes regarding funding in Canada and Scotland are referred to in a misleading manner. Pharmac has relied upon the minutes for advice regarding eculizumab and has drawn the minutes to the attention of submitters via a prominent link on the website page about the consultation. See http://www.pharmac.health.nz/news/item/proposal-to-decline-a-funding-application-for-eculizumab (accessed online 22 May 2012 and again 29 July 2013).

The PTAC minutes of August 2012 at para 3.11 discuss the drug's high cost and poor cost-effectiveness: "The Subcommittee noted that this is the reason why the Canadian Agency for Drugs and Technologies in Health (CADTH) and Scottish Medicines Consortium did not recommend it for use within their jurisdictions." Pharmac refers to the PTAC recommendations about cost and cost-effectiveness as part of its own reasoning in the consultation document. However neither PTAC, in those or their subsequent March 2013 minutes, nor Pharmac in their consultation document, make note of the fact that subsequent decisions in Canada have seen the drug funded in most parts of that country. There is also no reference to the Scottish government taking steps to set up a special fund for orphan drugs for rare diseases, in part because of the equity issues raised by the recommendations not to fund eculizumab, and have commenced funding patients there.

In failing to present the other side of the story about the position in Canada and Scotland, Pharmac will have misled many submitters as to funding decisions made in other jurisdictions.

NZORD considers these errors and misleading statements result in the consultation paper not suitably informing submitters. The errors seriously prejudice the interests of the patients who are seeking treatment with eculizumab and will have mislead potential submitters who might have relied wholly on the information Pharmac has provided when forming their views and making a decision on whether to provide a submission.

We believe Pharmac has a duty to correct the information and start the consultation afresh.

Please advise us as soon as possible what you intend to do to in response to this request. We are happy to discuss this with you further if that would assist.

Please note this email only focuses on those matters we consider require urgent attention to ensure Pharmac's consultation process is valid. This email does not describe all of NZORD's views on the consultation document. We consider other factors are also relevant to your decision (such as the right each patient has to health, and other community values). We intend to make a fuller submission (on a revised consultation document) in due course.

Yours sincerely,
john

John Forman
Executive Director, NZORD
New Zealand Organisation for Rare Disorders
PO Box 38-538, Wellington Mail Centre, 5045
228 Tinakori Rd, Thorndon, Wellington, 6011
New Zealand
Ph +64 [redacted]
Mob +64 [redacted]
Email [redacted]
Website: www.nzord.org.nz

From: Olivia Walley [redacted]
Sent: Sunday, 2 June 2013 8:59 a.m.
Subject: Submission in response to PHARMAC's consultation on Decision Critera.

To Whom it May Concern;

I support PNH patients in New Zealand in their fight to gain access to the life saving treatment Soliris.

I DO NOT support PHARMAC's proposal to decline funding for the Soliris treatment.

I support the PNH Support Association's proposition for Equity and Fairness which states:

i.
PHARMAC must return to the negotiating table with the supplier of the Soliris treatment

ii.
PHARMAC must negotiate in good faith toward funding Soliris for a minimum of 8 NZ PNH patients

iii.
PHARMAC must establish fair assessment criteria, based on expert advice from the international haematological community, to assess patient need for the Soliris treatment

iv.
PHARMAC must amend its Operating Policies and Procedures to acknowledge the right of rare disease patients to access life restoring and life saving treatments as in the specific example of the Soliris treatment

Yours faithfully

Olivia Walley

Sent from my iPhone

From: [redacted]
Sent: Tuesday, 30 July 2013 7:25 p.m.
To: eculizumabfeedback
Subject: Submission on consideration of SOLIRIS
Attachments: Pharmacs_intent_to_decline_Soliris_2013 submission.docx

Subject: **Submission to oppose PHARAMC'S proposal to decline a funding application for Soliris (eculizumab)**

To the appropriate members of PHARMAC,

I support the PNH patients in New Zealand in their fight to gain access to the lifesaving treatment Soliris.

I <u>DO NOT</u> support Pharmac's proposed intent to decline this treatment.

This is something I understand. My son, [redacted] endured an agonising decline and death from a rare lysosomal disease. Therapies were not available to him. I cared for him throughout his life and nursed him through to the moment of his death. He was [redacted].

When Pharamc takes this type of consultation to the public I should be able to rely on Pharmac to provide accurate and reliable information that will help guide responses from the community. But in 2011, you overstated the number of people affected with PNH which in turn over exaggerated the likely real cost of treatment. Pharmac staff reported to the Board that there would be up to 100 affected patients in New Zealand with Pompe disease. That treatment was declined, and it looks as though you intend to decline Soliris, again with dubious data as the basis for a decision.
This is not acceptable and you should withdraw this consultation because of the misleading information in it, which is likely to skew responses from the public.

These people have a right to life and our health system has a duty to address their needs in a fair and equitable way. When Pharmac places such emphasis on costs, cost-effectiveness and alternative use of the money, but does not address issues of rights, equity, fairness and community values; you are failing in your duty. As the District Health Boards have this duty, and Pharmac is acting as their purchasing agency, you should use the same decision criteria and priorities that they have.

I know all too well that people with rare diseases are disadvantaged because their condition *is* rare, and when treatments become available they are doubly affected by the high cost and the very small market due to small numbers. It is a denial of their right to health, and contrary to goals of "Equity of access, reducing inequalities and improving health outcomes for individuals and communities will guide our relationship and decision making", which are set out in the agreement between DHBs and Pharmac about how you decide things on their behalf.

Pharmac's assumption that "best health outcomes" as mentioned in its legislation, can be strongly associated with calculation of Quality Adjusted Life Years, is too narrow a view of what is "best", especially when Pharmac's calculations do not take into account non-health-sector costs. The decision criteria should include a broader range of considerations that are important to patients, so that the decisions are made in a patient-centred way.

Pharmac also has a responsibility for "health outcomes that are reasonably achievable". Your narrow perspective on technical assessment and budget management, to the exclusion of patient rights and interests from decisions, are an outcome that effectively discriminates against patients with rare diseases, and is not a reasonable outcome by any measure.

I do not support your intent to decline treatment for the PNH group or other patients where there are therapies for rare diseases.
PLEASE REGISTER MY SUBMISSION AND REMEMBER MY SON FOR WHOM THERE WAS NO HOPE. THE THERAPY YOU ARE CONSIDERING OFFERS MUCH MORE THAN HOPE, IT OFFERS THEM A LIFE.

[redacted]

From: patrick lundberg [redacted]
Sent: Monday, 17 June 2013 11:35 a.m.
To: eculizumabfeedback
Subject: Submission to PHARMAC's Proposal to decline a funding application for Soliris (eculizumab)

To Whom it May Concern;

I support PNH patients in New Zealand in their fight to gain access to the life saving treatment Soliris.

I DO NOT support PHARMAC's proposal to decline funding for the Soliris treatment.

I support the PNH Support Association's proposition for Equity and Fairness which states:

i. **PHARMAC must return to the negotiating table** with the supplier of the Soliris treatment

ii. **PHARMAC must negotiate in good faith** toward funding Soliris for a minimum of 8 NZ PNH patients

iii. **PHARMAC must establish fair assessment criteria**, based on expert advice from the international haematological community, to assess patient need for the Soliris treatment

iv. **PHARMAC must amend its Operating Policies and Procedures** to acknowledge the right of rare disease patients to access life restoring and life saving treatments as in the specific example of the Soliris treatment

I find it difficult to believe that I live in a country which would refuse support to sufferers of any disease, however rare. Given that a treatment is available for PNH I see it as the states obligation to ensure that those citizens who suffer from it be given access to that treatment. What other option could possibly be reasonable?

Yours faithfully

Patrick Lundberg

From: Paul Marquardt [redacted]
Sent: Tuesday, 30 July 2013 5:29 p.m.
To: eculizumabfeedback
Subject: Solaris - PNH

I support the PNH patients in New Zealand in their fight to gain access to the lifesaving treatment Soliris.

I **DO NOT** support Pharmac's proposed intent to decline this treatment.

- When Pharamc takes this type of consultation to the public I should be able to rely on Pharmac to provide accurate and reliable information that will help guide responses from the community. In this consultation you have overstated the number of people affected with PNH which in turn over exaggerates the likely real cost of treatment. You did this back in 2011 when Pharmac staff reported to the Board that there would be up to 100 affected patients in New Zealand with Pompe disease. That treatment was declined, and it looks as though you intend to decline Soliris, again with dubious data as the basis for a decision. This is not acceptable and you should withdraw this consultation because of the misleading information in it, which is likely to skew responses from the public.

- These patients have a right to life. Our health system has a duty to address their needs in a fair and equitable way. When Pharmac places so much emphasis on costs, cost-effectiveness and alternative use of the money, but does not address issues of rights, equity, fairness and community values; you are failing in your duty. The District Health Boards have this duty, and Pharmac is acting as their purchasing agency. You should use the same decision criteria and priorities that they have.

- Patients with rare diseases are disadvantaged because their condition is rare, and when treatments become available they are doubly affected by the high cost and the very small market due to small numbers. It is a denial of their right to health, and contrary to goals of "Equity of access, reducing inequalities and improving health outcomes for individuals and communities will guide our relationship and decision making", which are set out in the agreement between DHBs and Pharmac about how you decide things on their behalf.

- **Decision Criteria: The decision criteria used to assess medicines are not fair for those who are affected by rare diseases. There should be an additional layer of decision-making for rare diseases that do not fit the standard cost effectiveness threshold for large populations. These additional layers already exist in Australia, England, Scotland and other places around the world, because they have recognised that it's a <u>fair way</u> to deal with the disadvantage of rare diseases.**

- Pharmac's assumption that "best health outcomes" as mentioned in its legislation, can be strongly associated with calculation of Quality Adjusted Life Years, is too narrow a view of what is "best", especially when Pharmac's calculations do not take into account non-health-sector costs. The decision criteria should include a broader range of considerations that are important to patients, so that the decisions are made in a patient-centred way.

-

Pharmac also has a responsibility for "health outcomes that are reasonably achievable". Your narrow perspective on technical assessment and budget management, to the exclusion of patient rights and interests from decisions, are an outcome that effectively discriminates against patients with rare diseases, and is not a reasonable outcome by any measure.

- Pharmac often emphasises the "tough decision" approach that some will be funded and others not. That is too simplistic and can also be unfair, as it is in this case. There are very good reasons to adopt an equitable approach that spreads medicine funding across as many areas of health need as possible, so that no group is completely abandoned. That is the approach across a wide range of services in our health system and Pharmac should adopt a similar approach, rather than exclude and abandon certain groups.

I do not support your intent to decline treatment for the PNH group or other patients where there are therapies for rare diseases.

Paul and Fiona Marquardt
[redacted]

Sent from my iPad

From: Paul Jorgensen [redacted]
Sent: Monday, 24 June 2013 10:56 a.m.
To: eculizumabfeedback
Subject: Submission to PHARMAC's Proposal to decline a funding application for Soliris (eculizumab)

To Whom it May Concern;

I support PNH patients in New Zealand in their fight to gain access to the life saving treatment Soliris.

I DO NOT support PHARMAC's proposal to decline funding for the Soliris treatment.

I support the PNH Support Association's proposition for Equity and Fairness which states:

i. **PHARMAC must return to the negotiating table** with the supplier of the Soliris treatment

ii. **PHARMAC must negotiate in good faith** toward funding Soliris for a minimum of 8 NZ PNH patients

iii. **PHARMAC must establish fair assessment criteria**, based on expert advice from the international haematological community, to assess patient need for the Soliris treatment

iv. **PHARMAC must amend its Operating Policies and Procedures** to acknowledge the right of rare disease patients to access life restoring and life saving treatments as in the specific example of the Soliris treatment

Yours faithfully,
Paul Jorgensen

From: Pauline Wisker [redacted]
Sent: Monday, 3 June 2013 5:55 a.m.
To: eculizumabfeedback
Subject: Fw: Please

To whom it may concern.

I support PNH in New Zealand in their fight to gain access to the life saving treatment Soliris.

I DO NOT support Pharmac's proposal to decline funding for the Soliris treatment.

I support the PNH Support Association's proposition for Equity and Fairness which states:--

1. PHARMAC must return to the negotiating table with the supplier of the Soliris treatment.

2. PHARMAC must negotiate in good faith towards funding Soliris for a minimum of 8 PNH patients.

3. PHARMAC must establish fair assessment criteria, based on expert advice from the international haematological community, to assess patient need for the Soliris treatment.

4. PHARMAC must amend its Operating Policies and Procedures to acknowledge the right of rare disease patients to access life restoring and life saving treatments as in the specific example of the Soliris treatment.

Please have compassion. Would you refuse the opportunity of this life saving drug if one of your family was affected?

signed: Pauline Wisker.
signed: Derek Wisker.

From: [redacted]
Sent: Wednesday, 31 July 2013 7:59 a.m.
To: eculizumabfeedback
Subject: Submission
Attachments: pharmac submission.doc

Attached is my submission for Eculizumab to be on the approved medication list

Submission to Pharmac to fund the treatment of Eculizumab for PNH Sufferers.

This submission opposes the current PHARMAC proposal to decline funding for the treatment of eculizumab (Solaris) for PHN Sufferers

I am writing this submission in support of [redacted] who is a PNH (Paroxysmal Nocturnal Hemoglobinuria) sufferer. I have watched [redacted] through both good times and bad, and realise that without the right medication her life is in the balance. It is frustrating to know that the appropriate medication is available in 40 other countries.

[redacted] is an effervescent young woman who could potentially be cut down in the prime of her life. She lives each day to the fullest, but is always aware that long term plans might not be realised.

She has studied hard, qualified [redacted], and holds down full time employment where she is very well respected. However, her employment obligations are an additional stress when she is forced to take time off because her health dictates. She contributes to the wellbeing of her country, yet her country does not appreciate her input. It seems PHARMAC can play God and has decided she doesn't deserve the quality medication that other New Zealand citizens with a "common" disease have access to. Natalie did not choose to be in the "rare disease" category.

[redacted] she is extremely frustrated that she cannot access the medication that would remove the anxiety she involuntarily puts upon her family [redacted].

PHARMAC's refusal to fund eculizumab, has decided two options for [redacted]:

1. to just accept that her own country has turned its back on her. Continue to deteriorate and eventually.......die. A situation impossible to reconcile.

2. to leave the country that she calls home, the country she loves. It makes no sense at all to be forced to leave your family and friends and re-establish a new life right during a time when their support is most needed. A very difficult ask indeed.

We hear about the "brain drain". [redacted]'s loss to NZ would certainly contribute to that. Again, this makes no sense at all when it is completely avoidable.

It is clear that PHARMACs focus is on their bottom line. Unfortunately this is not delivering the outcomes they have been assigned. It's a sad situation when people's lives and health are measured in terms of dollars and cents. New Zealand CAN AFFORD to provide their citizens good health.

I implore PHARMAC to reconsider its decision not to fund eculizumab and make it available so that PNH sufferers can live a normal life with a normal life expectancy. The drug's efficacy is proven – there is no doubt about that. If funding medication for this rare disease does not fit within the current PHARMAC decision-making model, then either a modified or separate model is urgently required.

With respect and looking forward to a positive turnaround

[redacted]

From: Phil Harrington [redacted]
Sent: Wednesday, 12 June 2013 1:44 p.m.
To: eculizumabfeedback
Subject: Submission to PHARMAC's Proposal to decline a funding application for Soliris (eculizumab)

To Whom it May Concern;

I support PNH patients in New Zealand in their fight to gain access to the life saving treatment Soliris.

I DO NOT support PHARMAC's proposal to decline funding for the Soliris treatment.

I support the PNH Support Association's proposition for Equity and Fairness which states:

i. **PHARMAC must return to the negotiating table** with the supplier of the Soliris treatment

ii. **PHARMAC must negotiate in good faith** toward funding Soliris for a minimum of 8 NZ PNH patients

iii. **PHARMAC must establish fair assessment criteria**, based on expert advice from the international haematological community, to assess patient need for the Soliris treatment

iv. **PHARMAC must amend its Operating Policies and Procedures** to acknowledge the right of rare disease patients to access life restoring and life saving treatments as in the specific example of the Soliris treatment

I respect that PHARMAC must make hard decisions, however given the small numbers of affected persons suffering from PNH, contrasted to the vast number of people draining the public healthcare system through their own ignorance and arrogance, I expect PHARMAC to do the right thing.

Yours Sincerely,

Phil Harrington

From: philip wisker [redacted]
Sent: Sunday, 2 June 2013 2:01 p.m.
To: eculizumabfeedback; OPP Review
Subject: Submission to PHARMAC's Proposal to decline a funding application for Soliris (eculizumab)

To Whom it May Concern;

I support PNH patients in New Zealand in their fight to gain access to the life saving treatment Soliris.

I DO NOT support PHARMAC's proposal to decline funding for the Soliris treatment.

I support the PNH Support Association's proposition for Equity and Fairness which states:

i. **PHARMAC must return to the negotiating table** with the supplier of the Soliris treatment

ii. **PHARMAC must negotiate in good faith** toward funding Soliris for a minimum of 8 NZ PNH patients

iii. **PHARMAC must establish fair assessment criteria**, based on expert advice from the international haematological community, to assess patient need for the Soliris treatment

iv. **PHARMAC must amend its Operating Policies and Procedures** to acknowledge the right of rare disease patients to access life restoring and life saving treatments as in the specific example of the Soliris treatment

Please fund the drug, people could die?? you know the facts

Yours faithfully

Philip Wisker

From: Phillip Carlisle [redacted]
Sent: Tuesday, 30 July 2013 7:21 p.m.
To: eculizumabfeedback
Subject: Submission to PHARMAC's Proposal to decline a funding application for Soliris (eculizumab)

To Whom it May Concern;

I support PNH patients in New Zealand in their fight to gain access to the life saving treatment Soliris.

I DO NOT support PHARMAC's proposal to decline funding for the Soliris treatment.

I support the PNH Support Association's proposition for Equity and Fairness which states:

i. **PHARMAC must return to the negotiating table** with the supplier of the Soliris treatment

ii. **PHARMAC must negotiate in good faith** toward funding Soliris for a minimum of 8 NZ PNH patients

iii. **PHARMAC must establish fair assessment criteria**, based on expert advice from the international haematological community, to assess patient need for the Soliris treatment

iv. **PHARMAC must amend its Operating Policies and Procedures** to acknowledge the right of rare disease patients to access life restoring and life saving treatments as in the specific example of the Soliris treatment

Your decision will affect the lives of some really good friends of mine who deserve the right to the treatment

Yours faithfully

Phillip Carlisle & Maree Forsyth

From: [redacted]
Sent: Friday, 26 July 2013 10:32 a.m.
To: eculizumabfeedback
Subject: Submission to PHARMAC's Proposal to decline a funding application for Soliris (eculizumab)

To Whom it May Concern;

I support PNH patients in New Zealand in their fight to gain access to the life-saving treatment Soliris.

I DO NOT support PHARMAC's proposal to decline funding for the Soliris treatment.
I support the PNH Support Association's proposition for Equity and Fairness which states:

i. **PHARMAC must return to the negotiating table** with the supplier of the Soliris treatment

ii. **PHARMAC must negotiate in good faith** toward funding Soliris for a minimum of 8 NZ PNH patients
iii. **PHARMAC must establish fair assessment criteria**, based on expert advice from the international haematological community, to assess patient need for the Soliris treatment
iv. **PHARMAC must amend its Operating Policies and Procedures** to acknowledge the right of rare disease patients to access life restoring and life-saving treatments as in the specific example of the Soliris treatment

Yours faithfully,

Pip Sheehan

From: Chhean Khoun [redacted]
Sent: Monday, 29 July 2013 3:06 p.m.
To: eculizumabfeedback; OPP Review
Subject: PNHSAA_Submission to PHARMAC's Proposal to decline a funding application for Soliris (eculizumab)
Attachments: 20130729_PNHSAA_Letter to PHARMAC_[Final].docx; 20130729_PNHSAA_Letter to PHARMAC_F[inal].pdf

To whom it may concern,

On behalf of the PNH Support Association of Australia (PNHSAA), please find attached our letter (PDF and word formats) of support for the PNH Support Association of New Zealand (PNHSANZ), in their fight to have Soliris funded in New Zealand.

We thank you for your time and we look forward to your reply shortly.

Your faithfully,

Chhean Khoun
c/o PNHSAA

pnhsaa | PNH Support Association Australia Inc

Monday 29th July 2013

PHARMAC
PO Box 10-254
Wellington 6143
NEW ZEALAND

Email: eculizumabfeedback@pharmac.govt.nz
Email: opp@pharmac.govt.nz

To whom it may concern,

RE: Submission to PHARMAC's Proposal to decline a funding application for Soliris (eculizumab)

We are writing in support of the patients and the PNH Support Association in New Zealand (PNHSANZ), in their fight to gain access to the life saving treatment Soliris.

PNHSAA was established in 2009 to provide support and education for PNH patients, their families and friends in Australia. We work closely with the PNSANZ given how rare PNH is, and how much support our patients needs.

As you are ware, PNH is a rare, debilitating, potentially life threatening blood disorder defined by chronic red blood cell destruction. The destruction leads to fatigue, anemia and life threatening blood clots. Left untreated, PNH patients may face many life threatening complications such as major blood clots, organ damage, acute renal failure, reduced quality of life and possible death.

We also would like to highlight that the only effective treatment for PNH is a drug called Soliris®. Worldwide Clinical evidence published in respected Medical Journals have proven that Soliris® reduces chronic haemolysis (destruction of oxygen-carrying red blood cells) in almost 100% of PNH patients – an astounding efficacy rate never seen before for any treatment for any disease EVER. Patients who undergo this treatment responded well with increased quality of life and with an increased life expectancy to a normal person. Other life changing benefits includes little or no blood transfusion and less dependent on some medications.

In 9 December 2010, an announcement was made by the Australian Government for Soliris® to be funded through the Life Saving Drugs Program from 1 January 2011. Alexion Pharmaceutical gained regulatory approval from the US Food and Drug Administration (FDA) to market Soliris® in 2007. PNH patients have access to the Soliris in over 30 countries worldwide, including many in Europe, the UK, Japan, and the US and even Brazil since 2007. We would like to see this happening in New Zealand as soon as possible to be in line with the rest of the world.

Since Soliris was funded in Australia, we believe that a dramatic increase in life expectancy, improved overall quality of life, new found hope and possibilities amongst our patients and their loved ones. Soliris has changed the lives of our patients and we would like to see this happen to our fellow patients in NZ.

What would life be like for PNH patients living without Soliris in Australia? For some, this may not be possible, and for others anxiety, fear of the unknown future and life threatening risk such as clots. These are real issues and fear our follow patients are facing and living with everyday and we fully understand their concerns on the possibilities of living without Soliris.

As patients and committee members for the PNHSAA, we strongly agree with the above. Our lives before Soliris were put on hold. We experienced many complications which included severe stomach pains, declining liver and heat functions, fatigue, self conscious issues on how we looked and felt all the time, anxiety, fear of the future and unknown of PNH and very angry on why we were not able to do much more. As young professionals, our career and work hours and abilities were limited to our energy levels and were not able to take on leading roles or the opportunities available to us. This not only created self di-motivation at a professional level, it also affected our personal lives as well.

In sharing these views, we would like to highlight the real cost of PHARMAC's proposed decision to not fund Soliris:

- Unnecessary loss of life (median survival without treatment = 10 years)
- Unnecessary suffering (blood clots, fatigue, pain, inability to work)
- Permanent expatriation to one of the 40 other countries where Soliris can be accessed, including Australia
- A clear message of DISINTEREST from the NZ government to the international scientific and business communities working in the field of rare disease
- A clear message of DISINTEREST from the NZ government to patients, families and supporters of the New Zealand rare disease community (8% of people suffer from a rare disease at some stage in their life)

These are some of the desperate faces that are affected by PHARMAC's decision. We note that some are mother, father, brother or sister, niece and nephews to their loved ones who are at risk from the many complications from PNH, which includes death. We cannot let this happen to their loved ones and the people and future of New Zealand.

[redacted]

The PNHSAA and the people of Australia DO NOT support PHARMAC's proposal to decline funding for the Soliris treatment.

We support the PNH Support Association's proposition for Equity and Fairness which states:

- **PHARMAC must return to the negotiating table** with the supplier of the Soliris treatment
- **PHARMAC must negotiate in good faith** toward funding Soliris for a minimum of 8 NZ PNH patients
- **PHARMAC must establish fair assessment criteria**, based on expert advice from the international haematological community, to assess patient need for the Soliris treatment
- **PHARMAC must amend its Operating Policies and Procedures** to acknowledge the right of rare disease patients to access life restoring and life saving treatments as in the specific example of the Soliris treatment.

The Soliris treatment can be accessed via public health-systems in Australia, Europe, England, Canada, parts of the Middle East and many Asian countries. PHARMAC is unique in it's aggressive determination to not provide access to these highly effective, highly specialised treatments to New Zealand patients.

PNH patients are simply asking for their right to life to be respected. Provide these patients with access to a treatment which will allow them to live.

We thank you for your time and understanding and look forward to your reply shortly.

Yours faithfully,

PNH Support Association of Australia
PO BOX 472, South Yarra
VIC 3141
Australia

From: Poppy Miller [redacted]
Sent: Wednesday, 5 June 2013 2:07 p.m.
To: eculizumabfeedback; OPP Review
Subject: Soliris Funding

To Whom it May Concern;

I support PNH patients in New Zealand in their fight to gain access to the life saving treatment Soliris.

I DO NOT support PHARMAC's proposal to decline funding for the Soliris treatment.

I support the PNH Support Association's proposition for Equity and Fairness which states:

i. **PHARMAC must return to the negotiating table** with the supplier of the Soliris treatment

ii. **PHARMAC must negotiate in good faith** toward funding Soliris for a minimum of 8 NZ PNH patients

iii. **PHARMAC must establish fair assessment criteria**, based on expert advice from the international haematological community, to assess patient need for the Soliris treatment

iv. **PHARMAC must amend its Operating Policies and Procedures** to acknowledge the right of rare disease patients to access life restoring and life saving treatments as in the specific example of the Soliris treatment

Yours faithfully,

Poppy Miller

From: Rachel Dell [redacted]
Sent: Wednesday, 31 July 2013 6:43 p.m.
To: eculizumabfeedback
Subject: Submission opposing PHARMAC's Proposal to decline a funding application for Soliris (eculizumab)

To Whom it May Concern;

I SUPPORT PNH patients in New Zealand in their fight to gain access to the lifesaving treatment Soliris.

I OPPOSE PHARMAC's proposal to decline funding for the Soliris treatment.

I support the PNH Support Association's proposition for Equity and Fairness which states:

i. **PHARMAC must return to the negotiating table** with the supplier of the Soliris treatment

ii. **PHARMAC must negotiate in good faith** toward funding Soliris for a minimum of 8 NZ PNH patients

iii. **PHARMAC must establish fair assessment criteria**, based on expert advice from the international haematological community, to assess patient need for the Soliris treatment

iv. **PHARMAC must amend its Operating Policies and Procedures** to acknowledge the right of rare disease patients to access life restoring and lifesaving treatments as in the specific example of the Soliris treatment

My cousin, [redacted], from whom you will receive a submission, has PNH and lives overseas and is unlikely to ever be able to return home to NZ to live, as she wishes, if Soliris is unavailable to PNH patients. She has a lot to offer NZ society and economy, and to her New Zealand family who all miss her dearly. I believe PHARMAC needs to fund the treatment that can save and extend the lives of PNH patients. The treatment needs to be able to be considered by insurance companies, and at the moment, if I understand things correctly, it cannot because PHARMAC does not fund it at all. Patients, including my cousin, need to have life saving treatment available to them here in New Zealand, rather than receiving better treatment in countries that are not their homeland.

Yours faithfully

[redacted]

From: Rachel McShane [redacted]
Sent: Wednesday, 26 June 2013 11:00 a.m.
To: eculizumabfeedback
Subject: Fair medical funding

To Whom it May Concern;

I support PNH patients in New Zealand in their fight to gain access to the life saving treatment Soliris.

I DO NOT support PHARMAC's proposal to decline funding for the Soliris treatment.

I support the PNH Support Association's proposition for Equity and Fairness which states:

i. **PHARMAC must return to the negotiating table** with the supplier of the Soliris treatment

ii. **PHARMAC must negotiate in good faith** toward funding Soliris for a minimum of 8 NZ PNH patients

iii. **PHARMAC must establish fair assessment criteria**, based on expert advice from the international haematological community, to assess patient need for the Soliris treatment

iv. **PHARMAC must amend its Operating Policies and Procedures** to acknowledge the right of rare disease patients to access life restoring and life saving treatments as in the specific example of the Soliris treatment

All New Zealanders should have access to the medicines available to give quality and add length to their lives.

Yours faithfully

Rachel McShane

From: rachel chenisse z [redacted]
Sent: Monday, 3 June 2013 3:46 p.m.
To: OPP Review; eculizumabfeedback
Subject: Soliris in NZ

To Whom it May Concern;

I support PNH patients in New Zealand in their fight to gain access to the life saving treatment Soliris.

I DO NOT support PHARMAC's proposal to decline funding for the Soliris treatment.

I support the PNH Support Association's proposition for Equity and Fairness which states:

i.
PHARMAC must return to the negotiating table with the supplier of the Soliris treatment

ii.
PHARMAC must negotiate in good faith toward funding Soliris for a minimum of 8 NZ PNH patients

iii.
PHARMAC must establish fair assessment criteria, based on expert advice from the international haematological community, to assess patient need for the Soliris treatment

iv.
PHARMAC must amend its Operating Policies and Procedures to acknowledge the right of rare disease patients to access life restoring and life saving treatments as in the specific example of the Soliris treatment

Yours faithfully

Rachel Zacharias

Sent from my iPhone

From: Ralph Sutherland [redacted]
Sent: Thursday, 20 June 2013 6:34 a.m.
To: eculizumabfeedback
Subject: Subject: Submission to PHARMAC's Proposal to decline a funding application for Soliris (eculizumab)

To Whom it May Concern;

I support PNH patients in New Zealand in their fight to gain access to the life saving treatment Soliris.

I DO NOT support PHARMAC's proposal to decline funding for the Soliris treatment.

I support the PNH Support Association's proposition for Equity and Fairness which states:

i. **PHARMAC must return to the negotiating table with the supplier of the Soliris treatment**

ii. **PHARMAC must negotiate in good faith toward funding Soliris for a minimum of 8 NZ PNH patients**

iii. **PHARMAC must establish fair assessment criteria, based on expert advice from the international haematological community, to assess patient need for the Soliris treatment**

iv. **PHARMAC must amend its Operating Policies and Procedures to acknowledge the right of rare disease patients to access life restoring and life saving treatments as in the specific example of the Soliris treatment**

I would ask you to reconsider your decision to decline funding for this treatment, even though it is expensive, it would be a great way to display compassion to the 8 people in NZ who require it to survive!

Yours faithfully

--

Ralph Sutherland
Senior Pastor

Lifechurch
PALMERSTON NORTH

Ph:
Cell:
Fax:
Email: [redacted]
www.lifechurchpn.co.nz

590 Featherston St
PO Box 1031
Palmerston North 4440

From: [redacted]
Sent: Wednesday, 24 July 2013 11:41 a.m.
To: eculizumabfeedback
Cc: Lesley Murphy
Subject: Rare Voices Australia Ltd Letter to PHARMAC - decline funding application for Soliris (eculizumab)
Attachments: RVA letter to PHARMAC.pdf

Importance: High

Dear Sue Anne Yee,

RE: Submission to PHARMAC's proposal to decline a funding application for Soliris® (eculizumab)

Please find attached a letter from the Rare Voices Australia Ltd. with regard to PHARMAC's proposal to decline a funding application for Soliris®.
A paper version will be posted to you for your file.

I am coming to Wellington NZ to attend a meeting being conducted by the New Zealand Organisation for Rare Disorders.
I welcome an opportunity to discuss this further either face to face whilst I there next week or over the phone.

Kindest Regards,

Megan Fookes
EXECUTIVE DIRECTOR

Rare Voices Australia Ltd.
Suite 2 /3 The Postern
Castlecrag NSW 2068

P
M
E [redacted]
W www.rarevoices.org.au

rare voices
AUSTRALIA

The unified voice for all Australians living with a rare disease

rare voices
AUSTRALIA

Sue Anne Yee
Therapeutic Group Manager
PHARMAC
Post: PO Box 10 254, Wellington 6143

RE: Submission to PHARAMC'S proposal to decline a funding application for Soliris (eculizumab)

Rare Voices Australia is writing to express our grave concern at PHARMAC's proposal to decline a funding application for Soliris® (eculizumab) for the treatment of those living with Paroxysmal nocturnal hemoglobinuria (PNH). Rare Voices Australia Ltd (RVA), is a national 'peak body' organisation, giving a voice to an estimated 1.7 million Australians who live with a rare disease.

In Australia the independent advisory body; Pharmaceutical Benefits Advisory Committee (PBAC) concluded that eculizumab is an effective treatment for those living with PNH. PNH is a very rare and life-threatening blood disorder that causes premature red blood cell death. Its effects can include blood clots, kidney failure and other serious complications. Soliris® reduces the breakdown of red blood cells, the underlying cause of these complications, and therefore improves symptoms and reduces the risk of premature death or severe illness associated with PNH. Further information please read PBAC recommendation to the Australian Government dated 29 September 2010:
http://www.health.gov.au/internet/ministers/publishing.nsf/Content/BDA26C2952B5DCA2CA2577AD0000671A/$File/nr153.pdf

The lives of all of the people currently receiving Soliris® in this country have been transformed. They remain in the work force, leading full and productive lives. One woman has now set up and operates her own new business! These patients deserve the chance of a treatment that offers them an effective way of managing their disease.

There is absolutely no ethical fairness what so ever in discriminating against someone with a rare disease and yet offering equally or more expensive treatments to groups of people with more common diseases. How is this acceptable and not questioned by the members of your Committee?

One of the many benefits of funding this treatment is that it encourages the pharmaceutical industry to take up the challenge of getting researchers and companies interested in investing and developing new therapies.
There are considerable financial risks involved in funding research and development of new treatments and therapies for rare diseases. The pharmaceutical industry cannot be expected to continually fund and support research into rare diseases when the government will not fund effective treatments using very shaky ethical grounds as their reason for refusal.
The need for new and effective treatments to be made available to patients faced with life-threatening and life-altering diseases is blatantly obvious. People with rare diseases deserve the same opportunities to access new therapies and treatments as people with more common diseases such as; cancer, diabetes and heart disease.
People who have a rare disease such as PNH had absolutely no 'say in it'. They were simply born with their rare condition. Much of the New Zealand health budget is spent on preventable diseases and yet people born with congenital, genetic conditions and other rare diseases are largely ignored. This inequity needs to be addressed immediately.

The importance of flexibility in assessing medicines, taking into account the small cohort of patient numbers, the variability of disease progression and the factors mentioned above need addressing as matter of urgency.
Presently we are entering an era of personalised, genetic therapies and PHARMAC and Government need to recognise this and quickly develop more flexible and empathetic processes of assessment to provide a more fair and equitable program to those who need it.
Rare Voices Australia urges you to adopt Soliris® onto the Pharmaceutical Schedule, thus allowing people who live with PNH disease the chance to live a fulfilling life.
I welcome a meeting to talk this further with you.

I urge you to reconsider your proposal to decline a funding application which will in turn prevent the effective treatment for those with PNH in your country.
I will be in Wellington NZ on 31st July until 2nd August and would be more than happy to discuss this further with you in person or over the phone.

Kindest Regards,

M Fookes

Megan Fookes
Executive Director
Rare Voices Australia Ltd
[redacted]

From: [redacted]
Sent: Thursday, 30 May 2013 4:15 p.m.
To: eculizumabfeedback
Subject: Submission to PHARMAC's Proposal to decline a funding application for Soliris (eculizumab)

To Whom it May Concern;

I support PNH patients in New Zealand in their fight to gain access to the life saving treatment Soliris.

I DO NOT support PHARMAC's proposal to decline funding for the Soliris treatment.

I support the PNH Support Association's proposition for Equity and Fairness which states:

i. **PHARMAC must return to the negotiating table with the supplier of the Soliris treatment**

ii. **PHARMAC must negotiate in good faith toward funding Soliris for a minimum of 8 NZ PNH patients**

iii. **PHARMAC must establish fair assessment criteria, based on expert advice from the international haematological community, to assess patient need for the Soliris treatment**

iv. **PHARMAC must amend its Operating Policies and Procedures to acknowledge the right of rare disease patients to access life restoring and life saving treatments as in the specific example of the Soliris treatment**

I find it absolutely appalling that here in New Zealand people would sit back and watch people die when there is life saving medication for them. I have a son who has a rare disease and each day I watch him fight his battle to get up every morning and lead a life that so many others take for granted. These people, through no fault of there own are fighting to survive, they do not need the extra burden of knowing that their lives are not worth saving.

Yours sincerely

[redacted]

From: Rebecca Marshall [redacted]
Sent: Tuesday, 4 June 2013 10:29 a.m.
To: eculizumabfeedback
Subject: Submission to PHARMAC's Proposal to decline a funding application for Soliris (eculizumab)

To Whom it May Concern,

I support PNH patients in New Zealand in their fight to gain access to the life saving treatment Soliris.

I DO NOT support PHARMAC's proposal to decline funding for the Soliris treatment.

I support the PNH Support Association's proposition for Equity and Fairness which states:

i. PHARMAC must return to the negotiating table with the supplier of the Soliris treatment
ii. PHARMAC must negotiate in good faith toward funding Soliris for a minimum of 8 NZ PNH patients
iii. PHARMAC must establish fair assessment criteria, based on expert advice from the international haematological community, to assess patient need for the Soliris treatment
iv. PHARMAC must amend its Operating Policies and Procedures to acknowledge the right of rare disease patients to access life restoring and life saving treatments as in the specific example of the Soliris treatment

Please take notice of our petition, and give kiwis the chance to keep living!

Regards

Rebecca Marshall

From: Scott & Bex [redacted]
Sent: Friday, 31 May 2013 11:54 p.m.
To: eculizumabfeedback
Subject: Submission to PHARMAC's Proposal to decline a funding application for Soliris (eculizumab)

To Whom it May Concern;

I support PNH patients in New Zealand in their fight to gain access to the life saving treatment Soliris.

I DO NOT support PHARMAC's proposal to decline funding for the Soliris treatment.

I support the PNH Support Association's proposition for Equity and Fairness which states:

i. **PHARMAC must return to the negotiating table** with the supplier of the Soliris treatment

ii. **PHARMAC must negotiate in good faith** toward funding Soliris for a minimum of 8 NZ PNH patients

iii. **PHARMAC must establish fair assessment criteria**, based on expert advice from the international haematological community, to assess patient need for the Soliris treatment

iv. **PHARMAC must amend its Operating Policies and Procedures** to acknowledge the right of rare disease patients to access life restoring and life saving treatments as in the specific example of the Soliris treatment

Yours faithfully

Rebecca Stapleford.

From:	Robyn Spedding [redacted]
Sent:	Tuesday, 30 July 2013 7:09 p.m.
To:	eculizumabfeedback
Subject:	Declining treatment

To provide feedback, please submit it in writing by **Wednesday, 31 July 2013** to:

Sue Anne Yee
Therapeutic Group Manager
PHARMAC
Email: eculizumabfeedback@pharmac.govt.nz
Fax: 04 460 4995
Post: PO Box 10 254, Wellington 6143

Subject: **Submission to PHARAMC'S proposal to decline a funding application for Soliris (eculizumab)**

To whom it may concern:

I support the PNH patients in New Zealand in their fight to gain access to the lifesaving treatment Soliris.

I **DO NOT** support Pharmac's proposed intent to decline this treatment.

When Pharamc takes this type of consultation to the public I should be able to rely on Pharmac to provide accurate and reliable information that will help guide responses from the community. In this consultation you have overstated the number of people affected with PNH which in turn over exaggerates the likely real cost of treatment. You did this back in 2011 when Pharmac staff reported to the Board that there would be up to 100 affected patients in New Zealand with Pompe disease. That treatment was declined, and it looks as though you intend to decline Soliris, again with dubious data as the basis for a decision. This is not acceptable and you should withdraw this consultation because of the misleading information in it, which is likely to skew responses from the public.

These patients have a right to life. Our health system has a duty to address their needs in a fair and equitable way. When Pharmac places so much emphasis on costs, cost-effectiveness and alternative use of the money, but does not address issues of rights, equity, fairness and community values; you are failing in your duty. The District Health Boards have this duty, and Pharmac is acting as their purchasing agency. You should use the same decision criteria and priorities that they have.

Patients with rare diseases are disadvantaged because their condition is rare, and when treatments become available they are doubly affected by the high cost and the very small market due to small numbers. It is a denial of their right to health, and contrary to goals of "Equity of access, reducing inequalities and improving health outcomes for individuals and communities will guide our relationship and decision making", which are set out in the agreement between DHBs and Pharmac about how you decide things on their behalf.

Decision Criteria: The decision criteria used to assess medicines are not fair for those who are affected by rare diseases. There should be an additional layer of decision-making for rare diseases that do not fit the standard cost effectiveness threshold for large populations. These additional layers already exist in Australia, England, Scotland and other places around the world, because they have recognised that it's a fair way to deal with the disadvantage of rare diseases.

Pharmac's assumption that "best health outcomes" as mentioned in its legislation, can be strongly associated with calculation of Quality Adjusted Life Years, is too narrow a view of what is "best", especially when Pharmac's calculations do not take into account non-health-sector costs. The decision criteria should include a broader range of considerations that are important to patients, so that the decisions are made in a patient-centred way.

Pharmac also has a responsibility for "health outcomes that are reasonably achievable". Your narrow perspective on technical assessment and budget management, to the exclusion of patient rights and interests from decisions, are an outcome that effectively discriminates against patients with rare diseases, and is not a reasonable outcome by any measure.

Pharmac often emphasises the "tough decision" approach that some will be funded and others not. That is too simplistic and can also be unfair, as it is in this case. There are very good reasons to adopt an equitable approach that spreads medicine funding across as many areas of health need as possible, so that no group is completely abandoned. That is the approach across a wide range of services in our health system and Pharmac should adopt a similar approach, rather than exclude and abandon certain groups.

I do not support your intent to decline treatment for the PNH group or other patients where there are therapies for rare diseases.

From: [redacted]
Sent: Sunday, 2 June 2013 9:41 p.m.
To: eculizumabfeedback
Subject: Submission to PHARMAC's Proposal to decline a funding

To Whom it May Concern;

I support PNH patients in New Zealand in their fight to gain access to the life saving treatment Soliris.

I DO NOT support PHARMAC's proposal to decline funding for the Soliris treatment.

I support the PNH Support Association's proposition for Equity and Fairness which states:

i. **PHARMAC must return to the negotiating table with the supplier of the Soliris treatment**

ii. **PHARMAC must negotiate in good faith toward funding Soliris for a minimum of 8 NZ PNH patients**

iii. **PHARMAC must establish fair assessment criteria, based on expert advice from the international haematological community, to assess patient need for the Soliris treatment**

iv. **PHARMAC must amend its Operating Policies and Procedures to acknowledge the right of rare disease patients to access life restoring and life saving treatments as in the specific example of the Soliris treatment**

I am acting on behalf of a dear friend whose brother is suffering from PNH. They are amazing people and deserve every good thing that life can offer them. It is a real shame that [redacted] is unable to have this drug to enhance, enrich and lengthen his life simply because of the issue of funding.

Please reconsider this decision so that people like [redacted], and my very dear friend, his sister [redacted], can live their lives with one less stress to worry about.

Yours faithfully

[redacted]

From: [redacted]
Sent: Wednesday, 31 July 2013 8:57 a.m.
To: eculizumabfeedback
Subject: Solaris Funding

To Whom it May Concern;

I am writing as the parent of a child with a severe medical condition, but not one that requires Solaris. My son has severe [redacted] and is treated with [redacted]. This is not funded in many countries, and had he not had it available at home he would not be the normal [redacted] year old he is now. He runs, jumps, plays sports, and generally is treated and is as fit and healthy as other children. If we had not had it available he would have spent much of his [redacted]. He is very bright and we are looking forward to seeing the positive outcome he has on the world.

When I look at the people that are denied medicine for financial reasons it horrifies me. I feel sad if someone dies because of a disease with no medicines, but baffled that people are denied it when it exists. If you met a man in the desert who could survive if only you shared a little water, you would share wouldn't you? That is what it is to be human.

Look at the profits, then tell us actually what the drug is worth. You've already done the research, take that out of the budget. Would you pay this much to save your own child's life? Is it really worth denying these people access to a medicine that can help a disease that they have acquired through no fault of their own? Access to solaris is a drop in the financial ocean for your company but will positively affect not only the 22 people, but their families, friends and communities.

I hope your conscience outweighs the bottom line.

Best Wishes,

[redacted]
Awesomeness International

[redacted]

www.rhysdarby.com
www.awesomenesscomedy.com
www.facebook.com/rhysdarby
www.twitter.com/rosiecd

From: Ross Merriman [redacted]
Sent: Thursday, 18 July 2013 9:39 p.m.
To: eculizumabfeedback
Subject: Life Saving Medication

I believe this medication should be funded or these lovely people may not survive!!! Please help them.

Sent from my iPad

From: Emilia Roxana Lupu [redacted]
Sent: Thursday, 6 June 2013 8:58 a.m.
To: eculizumabfeedback; OPP Review
Subject: Submission in response to PHARMAC's consultation on Decision Critera

To Whom it May Concern;

I support PNH patients in New Zealand in their fight to gain access to the life saving treatment Soliris.

I DO NOT support PHARMAC's proposal to decline funding for the Soliris treatment.

I support the PNH Support Association's proposition for Equity and Fairness which states:

i. **PHARMAC must return to the negotiating table** with the supplier of the Soliris treatment

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iii. **PHARMAC must establish fair assessment criteria**, based on expert advice from the international haematological community, to assess patient need for the Soliris treatment

iv. **PHARMAC must amend its Operating Policies and Procedures** to acknowledge the right of rare disease patients to access life restoring and life saving treatments as in the specific example of the Soliris treatment

It is a disgrace that we pay for years for healthy people staying at home on doll but we cannot subsidy sick people and children to get the required treatment.

Yours faithfully,

Roxana Lupu

From: Everlasting [redacted]
Sent: Tuesday, 16 July 2013 4:38 p.m.
To: eculizumabfeedback
Subject: Make Soliris Available to PNH patients in New Zealand

---

Re: Submission opposing PHARMAC's Proposal to decline a funding application for Soliris (eculizumab)

To Whom it May Concern;

I SUPPORT PNH patients in New Zealand in their fight to gain access to the lifesaving treatment Soliris.

I OPPOSE PHARMAC's proposal to decline funding for the Soliris treatment.

I support the PNH Support Association's proposition for Equity and Fairness which states:

i. **PHARMAC must return to the negotiating table** with the supplier of the Soliris treatment

ii. **PHARMAC must negotiate in good faith** toward funding Soliris for a minimum of 8 NZ PNH patients

iii. **PHARMAC must establish fair assessment criteria,** based on expert advice from the international haematological community, to assess patient need for the Soliris treatment

iv. **PHARMAC must amend its Operating Policies and Procedures** to acknowledge the right of rare disease patients to access life restoring and lifesaving treatments as in the specific example of the Soliris treatment

Yours faithfully

Mrs. Ruth Spear

From: Ryan Cartlidge [redacted]
Sent: Wednesday, 17 July 2013 12:36 p.m.
To: eculizumabfeedback; OPP Review
Subject: Submission opposing PHARMAC's Proposal to decline a funding application for Soliris (eculizumab)

To Whom it May Concern;

I SUPPORT PNH patients in New Zealand in their fight to gain access to the lifesaving treatment Soliris.
I OPPOSE PHARMAC's proposal to decline funding for the Soliris treatment.
I support the PNH Support Association's proposition for Equity and Fairness which states:

i. **PHARMAC must return to the negotiating table** with the supplier of the Soliris treatment
ii. **PHARMAC must negotiate in good faith** toward funding Soliris for a minimum of 8 NZ PNH patients
iii. **PHARMAC must establish fair assessment criteria**, based on expert advice from the international haematological community, to assess patient need for the Soliris treatment

iv. **PHARMAC must amend its Operating Policies and Procedures** to acknowledge the right of rare disease patients to access life restoring and lifesaving treatments as in the specific example of the Soliris treatment

(please include your personal statements here)

Yours faithfully

Ryan Cartlidge

From: Salomina Tijkken [redacted]
Sent: Friday, 21 June 2013 9:10 a.m.
To: eculizumabfeedback
Subject: Submission to PHARMAC's Proposal to decline a funding application for Soliris (eculizumab)

To Whom it May Concern;

I support PNH patients in New Zealand in their fight to gain access to the life saving treatment Soliris.

I DO NOT support PHARMAC's proposal to decline funding for the Soliris treatment.

I support the PNH Support Association's proposition for Equity and Fairness which states:

i. **PHARMAC must return to the negotiating table** with the supplier of the Soliris treatment

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iii. **PHARMAC must establish fair assessment criteria,** based on expert advice from the international haematological community, to assess patient need for the Soliris treatment

iv. **PHARMAC must amend its Operating Policies and Procedures** to acknowledge the right of rare disease patients to access life restoring and life saving treatments as in the specific example of the Soliris treatment

Yours faithfully

Salomina Tijkken-Batlajeri

From: sam hall [redacted]
Sent: Friday, 31 May 2013 8:44 p.m.
To: eculizumabfeedback
Subject: Solaris

Its unacceptable to let people die, especially when there is life saving treatment available. Shame on Pharmacy and shame on New Zealand.

From: [redacted]
Sent: Tuesday, 9 July 2013 4:26 p.m.
To: eculizumabfeedback
Subject: Submission to PHARMAC's Proposal to decline a funding application for Soliris (eculizumab)

To Whom it May Concern;
I support PNH patients in New Zealand in their fight to gain access to the life saving treatment Soliris.

I DO NOT support PHARMAC's proposal to decline funding for the Soliris treatment.

I support the PNH Support Association's proposition for Equity and Fairness which states:

i. **PHARMAC must return to the negotiating table with the supplier of the Soliris treatment**

ii. **PHARMAC must negotiate in good faith toward funding Soliris for a minimum of 8 NZ PNH patients**

iii. **PHARMAC must establish fair assessment criteria, based on expert advice from the international** haematological community, to assess patient need for the Soliris treatment

iv. **PHARMAC must amend its Operating Policies and Procedures** to acknowledge the right of rare disease patients to access life restoring and life saving treatments as in the specific example of the Soliris treatment

I am writing this because I am a close family friend of a PNH patient ([redacted]*), I can't express how shocked I was to read that their is a treatment out there that can help her yet* [redacted] *has to choose between treatment or never returning to live in her home country.*

I have been a friend of [redacted] *and her family for over* [redacted] *years and grew up with* [redacted]*, they are a close family even though they are currently living in all corners of the world. I know that* [redacted] *mother has looked forward to the day that* [redacted] *and her siblings will return to live closer to her. The fact that there is a solution and one that doesn't have to come at such a huge cost to both* [redacted] *and her family (not to mention her partner who is also looking forward to returning to NZ to be closer to his parents) seems insane to me.*

I hope that this can be re-looked at not only for [redacted] *but for all PNH sufferers, there are many diseases with no treatment right now but there IS something that can be done here in NZ for them, so please re-consider this.*

Yours faithfully

[redacted]

From: Sapphire Jean Ruth Lukupa [redacted]
Sent: Monday, 3 June 2013 5:25 p.m.
To: eculizumabfeedback
Subject: Soliris- Please help

Please,

Please return to the negotiating table with the supplier of the Soliris treatment, anything you do to try and change the lives of the(my) loved ones that suffer from the effects of not being able to afford the treatment would be just amazing!!! Or if you could source funding from other companies? Anything you can do within your power would make a real difference- every life is worth saving.

Kind Regards,

Jean

P.S - Stranger, I would do it for you...

From: OPP Review
Sent: Wednesday, 5 June 2013 10:50 a.m.
To: eculizumabfeedback
Subject: FW: Submission in response to PHARMAC's consultation on Decision Criteria.

[SEEMail]

From: Sapphire Lukupa [redacted]
Sent: Monday, 3 June 2013 5:29 p.m.
To: OPP Review
Subject: Submission in response to PHARMAC's consultation on Decision Criteria.

To Whom it May Concern;

I support PNH patients in New Zealand in their fight to gain access to the life saving treatment Soliris.

I DO NOT support PHARMAC's proposal to decline funding for the Soliris treatment.

I support the PNH Support Association's proposition for Equity and Fairness which states:

i. **PHARMAC must return to the negotiating table** with the supplier of the Soliris treatment

ii. **PHARMAC must negotiate in good faith** toward funding Soliris for a minimum of 8 NZ PNH patients

iii. **PHARMAC must establish fair assessment criteria**, based on expert advice from the international haematological community, to assess patient need for the Soliris treatment

iv. **PHARMAC must amend its Operating Policies and Procedures** to acknowledge the right of rare disease patients to access life restoring and life saving treatments as in the specific example of the Soliris treatment

Please do what ever you can to help, every life is worth saving.

Yours faithfully

S. Lukupa

To: OPP Review[opp@Pharmac.govt.nz]
From: sarah jones
Sent: Mon 29/07/2013 7:10:10 a.m
Importance: Normal
Subject: Submission to PHARMAC'S proposal to decline a funding application for Soliris (eculizumab)
MAIL_RECEIVED: Mon 29/07/2013 7:10:14 a.m

To whom it may concern:

I support the PNH patients in New Zealand in their fight to gain access to the l...lifesaving treatment Soliris.

I DO NOT support Pharmac's proposed intent to decline this treatment. I also believe it would be a backward step for New Zealand as a whole.

New Zealand is a civilized democracy. We are part of the United Nations and pride ourselves on out human rights record. We hold our own on the world stage in every area of excellence in spite of being a small population. We have earned respect! Our public health system and our medicines purchasing agency (Pharmac), are held up as models by which other nations strive to aspire. However, on the issue of treatment for rare diseases, these other nations take the lead while we lethargically drag our heels. We are told by Pharmac that the issue is about cost and cost effectiveness, and there is no doubt that the new, innovative medicines are costly, not just to New Zealand, but to the international market. However, in spite of these high costs, other cash strapped nations appear to be meeting the challenge. This, in itself, makes our refusal to treat even more difficult to justify.

If we take the upholding of human rights seriously, we would agree that New Zealand citizens have a right to life. Hence, we have a moral obligation to ensure that everyone has access to life sustaining treatment when such treatments become available. No clinical population should ever be completely abandoned in our society. The notion that patients with rare diseases can be ignored because the costs are higher than average, is callous and inhumane. I recognize that it must be difficult at times to balance the books, but it would be morally wrong to achieve this by denying treatment to this small, select group of patients.

Patients with rare diseases are already disadvantaged because of deteriorating health and all that this entails; they should not be subjected to the added pressures of having to fight for the health benefits that most other New Zealanders take for granted.

As I see it, a far more compassionate approach would be for Pharmac to negotiate lower prices with drug companies, which incidentally is the precise expertise upon which Pharmac has built its international reputation. Come on Pharmac! Do the right thing! Take up this challenge and fund these drugs!

I do not support your intent to decline treatment for the PNH group or other patients where there are therapies for rare diseases.

Sincerely,
Sarah Jones

From: Sarah Papageorgiou [redacted]
Sent: Wednesday, 5 June 2013 4:05 p.m.
To: eculizumabfeedback
Subject: Submission to PHARMAC's Proposal to decline a funding application for Soliris (eculizumab)

To Whom it May Concern;

I support PNH patients in New Zealand in their fight to gain access to the life saving treatment Soliris.

I DO NOT support PHARMAC's proposal to decline funding for the Soliris treatment.

I support the PNH Support Association's proposition for Equity and Fairness which states:

i. **PHARMAC must return to the negotiating table** with the supplier of the Soliris treatment

ii. **PHARMAC must negotiate in good faith** toward funding Soliris for a minimum of 8 NZ PNH patients

iii. **PHARMAC must establish fair assessment criteria**, based on expert advice from the international haematological community, to assess patient need for the Soliris treatment

iv. **PHARMAC must amend its Operating Policies and Procedures** to acknowledge the right of rare disease patients to access life restoring and life saving treatments as in the specific example of the Soliris treatment

Yours faithfully

Sarah Papageorgiou

[redacted]

From: Sarah-Jayne Doran [redacted]
Sent: Wednesday, 24 July 2013 7:00 p.m.
To: eculizumabfeedback
Subject: Submission to PHARMAC's Proposal to decline a funding application for Soliris (eculizumab)

To Whom it May Concern;

I support PNH patients in New Zealand in their fight to gain access to the life saving treatment Soliris.
I DO NOT support PHARMAC's proposal to decline funding for the Soliris treatment.
I support the PNH Support Association's proposition for Equity and Fairness which states:
PHARMAC must return to the negotiating table with the supplier of the Soliris treatment
PHARMAC must negotiate in good faith toward funding Soliris for a minimum of 8 NZ PNH patients
PHARMAC must establish fair assessment criteria, based on expert advice from the international haematological community, to assess patient need for the Soliris treatment
PHARMAC must amend its Operating Policies and Procedures to acknowledge the right of rare disease patients to access life restoring and life saving treatments as in the specific example of the Soliris treatment

Please consider the cost of a human life, how can a life be measured in financial terms? What sort of society are we if we allow people to suffer and die young because it costs too much money? Please treat this as if it was your loved one.

Yours faithfully

Sarah Seaholme

From: Sarah Tapsell [redacted]
Sent: Wednesday, 31 July 2013 8:29 p.m.
To: eculizumabfeedback
Subject: I DO NOT support PHARMAC's proposal to decline funding for the Soliris treatment

To Whom it May Concern;

I support PNH patients in New Zealand in their fight to gain access to the life saving treatment Soliris.

I DO NOT support PHARMAC's proposal to decline funding for the Soliris treatment.

I support the PNH Support Association's proposition for Equity and Fairness which states:

i.
PHARMAC must return to the negotiating table with the supplier of the Soliris treatment

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iii.
PHARMAC must establish fair assessment criteria, based on expert advice from the international haematological community, to assess patient need for the Soliris treatment

iv.
PHARMAC must amend its Operating Policies and Procedures to acknowledge the right of rare disease patients to access life restoring and life saving treatments as in the specific example of the Soliris treatment

Yours faithfully

Sarah Tapsell

From: Sarah [redacted]
Sent: Sunday, 2 June 2013 10:23 a.m.
To: eculizumabfeedback
Subject: Submission to PHARMAC's Proposal to decline a funding application for Soliris (eculizumab)

To Whom it May Concern;

I support PNH patients in New Zealand in their fight to gain access to the life saving treatment Soliris.

I DO NOT support PHARMAC's proposal to decline funding for the Soliris treatment.

I support the PNH Support Association's proposition for Equity and Fairness which states:

i. **PHARMAC must return to the negotiating table** with the supplier of the Soliris treatment

ii. **PHARMAC must negotiate in good faith** toward funding Soliris for a minimum of 8 NZ PNH patients

iii. **PHARMAC must establish fair assessment criteria,** based on expert advice from the international haematological community, to assess patient need for the Soliris treatment

iv. **PHARMAC must amend its Operating Policies and Procedures** to acknowledge the right of rare disease patients to access life restoring and life saving treatments as in the specific example of the Soliris treatment

Yours faithfully

Sarah van Herpt

From: Sararose Brown [redacted]
Sent: Tuesday, 4 June 2013 8:02 p.m.
To: eculizumabfeedback
Subject: Submission to PHARMAC's Proposal to decline a funding application for Soliris (eculizumab)
Attachments: proposition_equity_and_fairness_-_email_submission_template.doc

04/06/13

To Whom it May Concern;

I support PNH patients in New Zealand in their fight to gain access to the life saving treatment Soliris.

I DO NOT support PHARMAC's proposal to decline funding for the Soliris treatment.

I support the PNH Support Association's proposition for Equity and Fairness which states:

i. **PHARMAC must return to the negotiating table** with the supplier of the Soliris treatment

ii. **PHARMAC must negotiate in good faith** toward funding Soliris for a minimum of 8 NZ PNH patients

iii. **PHARMAC must establish fair assessment criteria**, based on expert advice from the international haematological community, to assess patient need for the Soliris treatment

iv. **PHARMAC must amend its Operating Policies and Procedures** to acknowledge the right of rare disease patients to access life restoring and life saving treatments as in the specific example of the Soliris treatment

Yours faithfully

Sararose Brown

[redacted]

From: Scott Mills [redacted]
Sent: Saturday, 1 June 2013 8:01 a.m.
To: eculizumabfeedback
Subject: Soliris

To Whom it May Concern;
I support PNH patients in New Zealand in their fight to gain access to the life saving treatment Soliris.
I DO NOT support PHARMAC's proposal to decline funding for the Soliris treatment.
I support the PNH Support Association's proposition for Equity and Fairness which states:
PHARMAC must return to the negotiating table with the supplier of the Soliris treatment
PHARMAC must negotiate in good faith toward funding Soliris for a minimum of 8 NZ PNH patients
PHARMAC must establish fair assessment criteria, based on expert advice from the international haematological community, to assess patient need for the Soliris treatment
PHARMAC must amend its Operating Policies and Procedures to acknowledge the right of rare disease patients to access life restoring and life saving treatments as in the specific example of the Soliris treatment

Please consider this proposal. Health care is about caring.......
Give these people a quality of life that normal people enjoy.
Thank you.

Yours faithfully

Scott Mills

From: Inky Sean O'Sullivan [redacted]
Sent: Tuesday, 25 June 2013 8:40 p.m.
To: eculizumabfeedback
Subject: Submission to PHARMAC's Proposal to decline a funding application for Soliris (eculizumab)

To Whom it May Concern;

I support PNH patients in New Zealand in their fight to gain access to the life saving treatment Soliris.

I DO NOT support PHARMAC's proposal to decline funding for the Soliris treatment.

I support the PNH Support Association's proposition for Equity and Fairness which states:

i. **PHARMAC must return to the negotiating table** with the supplier of the Soliris treatment

ii. **PHARMAC must negotiate in good faith** toward funding Soliris for a minimum of 8 NZ PNH patients

iii. **PHARMAC must establish fair assessment criteria**, based on expert advice from the international haematological community, to assess patient need for the Soliris treatment

iv. **PHARMAC must amend its Operating Policies and Procedures** to acknowledge the right of rare disease patients to access life restoring and life saving treatments as in the specific example of the Soliris treatment

Yours faithfully

Sean and Ingrid O'Sullivan

From: Shannon Gantley [redacted]
Sent: Tuesday, 30 July 2013 7:26 p.m.
To: eculizumabfeedback
Subject: Submission to PHARAMC'S proposal to decline a funding application for Soliris (eculizumab)

To whom it may concern:
I support the PNH patients in New Zealand in their fight to gain access to the lifesaving treatment Soliris.
I DO NOT support Pharmac's proposed intent to decline this treatment
When Pharmac take this type of consultation to the public I should be able to rely on Pharmac to provide accurate and reliable information that will help guide responses from the community. In this consultation you have overstated the number of people affected with PNH which in turn over exaggerates the likely real cost of treatment. You did this back in 2011 when Pharmac staff reported to the Board that there would be up to 100 affected patients in New Zealand with Pompe disease. That treatment was declined, and it looks as though you intend to decline Soliris, again with dubious data as the basis for a decision. This is not acceptable and you should withdraw this consultation because of the misleading information in it, which is likely to skew responses from the public. .

These patients have a right to life. Our health system has a duty to address their needs in a fair and equitable way. When Pharmac places so much emphasis on costs, cost-effectiveness and alternative use of the money, but does not address issues of rights, equity, fairness and community values, you are failing in your duty. The District Health Boards have this duty, and Pharmac is acting as their purchasing agency. You should use the same decision criteria and priorities that they have.

Patients with rare diseases are disadvantaged because their condition is rare, and when treatments become available they are doubly affected by the high cost and the very small market due to small numbers. It is a denial of their right to health, and contrary to goals of "Equity of access, reducing inequalities and improving health outcomes for individuals and communities will guide our relationship and decision making", which are set out in the agreement between DHBs and Pharmac about how you decide things on their behalf.

Decision Criteria: The decision criteria used to assess medicines are not fair for those who are affected by rare diseases. There should be an additional layer of decision-making for rare diseases that do not fit the standard cost effectiveness threshold for large populations. These additional layers already exist in Australia, England, Scotland and other places around the world, because they have recognised that it's a fair way to deal with the disadvantage of rare diseases.

Pharmac's assumption that "best health outcomes" as mentioned in its legislation, can be strongly associated with calculation of Quality Adjusted Life Years, is too narrow a view of what is "best", especially when Pharmac's calculations do not take into account non-health-sector costs. The decision criteria should include a broader range of considerations that are important to patients, so that the decisions are made in a patient-centred way.
Pharmac also has a responsibility for "health outcomes that are reasonably achievable". Your narrow perspective on technical assessment and budget management, to the exclusion of patient rights and interests from decisions, are an outcome that effectively discriminates against patients with rare diseases, and is not a reasonable outcome by any measure.
Pharmac often emphasises the "tough decision" approach that some will be funded and others not. That is too simplistic and can also be unfair, as it is in this case. There are very good reasons to adopt an equitable approach that spreads medicine funding across as many areas of health need as possible so that no group is

completely abandoned. That is the approach across a wide range of services in our health system and Pharmac should adopt a similar approach, rather than exclude and abandon certain groups.
I do not support your intent to decline treatment for this group or other patients where there are therapies for rare diseases.

From: [redacted]
Sent: Tuesday, 4 June 2013 7:59 p.m.
To: eculizumabfeedback
Subject: Submission to PHARMAC's Proposal to decline a funding application for Soliris (eculizumab)

To Whom it May Concern;

I support PNH patients in New Zealand in their fight to gain access to the life saving treatment Soliris.

I DO NOT support PHARMAC's proposal to decline funding for the Soliris treatment.

I support the PNH Support Association's proposition for Equity and Fairness which states:

i. **PHARMAC must return to the negotiating table with the supplier of the Soliris treatment**

ii. **PHARMAC must negotiate in good faith toward funding Soliris for a minimum of 8 NZ PNH patients**

iii. **PHARMAC must establish fair assessment criteria, based on expert advice from the international haematological community, to assess patient need for the Soliris treatment**

iv. **PHARMAC must amend its Operating Policies and Procedures to acknowledge the right of rare disease patients to access life restoring and life saving treatments as in the specific example of the Soliris treatment**

I am a friend of [redacted] *family. I have seen first hand how his condition affects everyone around him, it is heartbreaking to watch. I just can not understand how anyone can sit back and 'play god' by not funding this medication for him that will save his life. PLEASE PLEASE PLEASE give this family what they need.. save* [redacted] *life.*

Yours faithfully

[redacted]

From: Sharelle Richardson [redacted]
Sent: Tuesday, 4 June 2013 10:54 a.m.
To: eculizumabfeedback

http://www.pnhsanz.org.nz/uploads/1/1/5/7/11570630/proposition_equity_and_fairness_-_email_submission_template.doc

☐ Sent via iPhone →📱

From: Sharon [redacted]
Sent: Tuesday, 30 July 2013 2:55 p.m.
To: eculizumabfeedback
Subject: Submission to PHARAMC'S proposal to decline a funding application for Soliris (eculizumab)
Attachments: Pharmacs_intent_to_decline_Soliris_2013.docx

To Sue Anne Yee

Attached please find my submission.

Kind regards

Sharon Kortas

To provide feedback, please submit it in writing by **Wednesday, 31 July 2013** to:

Sue Anne Yee
Therapeutic Group Manager
PHARMAC

Email:
Fax: 04 460 4995
Post: PO Box 10 254, Wellington 6143

Subject: **Submission to PHARAMC'S proposal to decline a funding application for Soliris (eculizumab)**

To whom it may concern:

I support the PNH patients in New Zealand in their fight to gain access to the lifesaving treatment Soliris.

I **DO NOT** support Pharmac's proposed intent to decline this treatment.

- When Pharamc takes this type of consultation to the public I should be able to rely on Pharmac to provide accurate and reliable information that will help guide responses from the community. In this consultation you have overstated the number of people affected with PNH which in turn over exaggerates the likely real cost of treatment. You did this back in 2011 when Pharmac staff reported to the Board that there would be up to 100 affected patients in New Zealand with Pompe disease. That treatment was declined, and it looks as though you intend to decline Soliris, again with dubious data as the basis for a decision. This is not acceptable and you should withdraw this consultation because of the misleading information in it, which is likely to skew responses from the public.

- These patients have a right to life. Our health system has a duty to address their needs in a fair and equitable way. When Pharmac places so much emphasis on costs, cost-effectiveness and alternative use of the money, but does not address issues of rights, equity, fairness and community values; you are failing in your duty. The District Health Boards have this duty, and Pharmac is acting as their purchasing agency. You should use the same decision criteria and priorities that they have.

- Patients with rare diseases are disadvantaged because their condition is rare, and when treatments become available they are doubly affected by the high cost and the very small market due to small numbers. It is a denial of their right to health, and contrary to goals of "Equity of access, reducing inequalities and improving health outcomes for individuals and communities will guide our relationship and decision making", which are set out in the agreement between DHBs and Pharmac about how you decide things on their behalf.

- **Decision Criteria:** The decision criteria used to assess medicines are not fair for those who are affected by rare diseases. There should be an additional layer of decision-making for rare diseases that do not fit the standard cost effectiveness threshold for large populations. These additional layers already exist in Australia, England, Scotland and other places around the world, because they have recognised that it's a fair way to deal with the disadvantage of rare diseases.

- Pharmac's assumption that "best health outcomes" as mentioned in its legislation, can be strongly associated with calculation of Quality Adjusted Life Years, is too narrow a view of what is "best", especially when Pharmac's calculations do not take into account non-health-sector costs. The decision criteria should include a broader range of considerations that are important to patients, so that the decisions are made in a patient-centred way.

- Pharmac also has a responsibility for "health outcomes that are reasonably achievable". Your narrow perspective on technical assessment and budget management, to the exclusion of patient rights and interests from decisions, are an outcome that effectively discriminates against patients with rare diseases, and is not a reasonable outcome by any measure.

- Pharmac often emphasises the "tough decision" approach that some will be funded and others not. That is too simplistic and can also be unfair, as it is in this case. There are very good reasons to adopt an equitable approach that spreads medicine funding across as many areas of health need as possible, so that no group is completely abandoned. That is the approach across a wide range of services in our health system and Pharmac should adopt a similar approach, rather than exclude and abandon certain groups.

I do not support your intent to decline treatment for the PNH group or other patients where there are therapies for rare diseases.

Sharon Kortas

[redacted]

From: Shelley Roberts [redacted]
Sent: Wednesday, 31 July 2013 11:44 a.m.
To: eculizumabfeedback; OPP Review
Subject: Funding for Soliris
Attachments: ______________proposition_equity_and_fairness_-_email_submission_template.doc
x

To Whom it May Concern;

I support PNH patients in New Zealand in their fight to gain access to the life saving treatment Soliris.

I DO NOT support PHARMAC's proposal to decline funding for the Soliris treatment.

I support the PNH Support Association's proposition for Equity and Fairness which states:

i. **PHARMAC must return to the negotiating table** with the supplier of the Soliris treatment

ii. **PHARMAC must negotiate in good faith** toward funding Soliris for a minimum of 8 NZ PNH patients

iii. **PHARMAC must establish fair assessment criteria,** based on expert advice from the international haematological community, to assess patient need for the Soliris treatment

iv. **PHARMAC must amend its Operating Policies and Procedures** to acknowledge the right of rare disease patients to access life restoring and life saving treatments as in the specific example of the Soliris treatment

I am personally attempting to fund the likes of [redacted] myself, as I am aware of his plight, have seen his photo, and cannot bare to know that my decision not to help him could result in his death. I can only give a couple of dollars a week, but I personally could never say no to a New Zealand child being funded and have his life saved. New Zealanders don't want this. I know you see this treatment as experimental, but really there is no other option for these patients. It's this drug or death. How can it be that in NZ, our people, particularly our children, don't get the medication they need to live? Please review this decision.

Yours faithfully

Shelley Urlich

[redacted]

From: [redacted]
Sent: Monday, 29 July 2013 8:23 p.m.
To: eculizumabfeedback
Subject: Pharmac
Attachments: Submission to Parmac.docx

Please consider the attached submission.
Our family live on a knife edge every day.
Regards
[redacted]

This submission opposes the current PHARMAC proposal to decline funding for the treatment eculizumab (soliris)

My niece [redacted] lives with Paroxysmal Nocturnal Hemoglobinuria (PNH) and funding for the drug eculizumab is the only key to giving her a normal life expectancy. I am at a loss as to why PHARMAC would sentence my niece and others living with the disease to such a difficult (and potentially shortened) life.

[redacted] The pain attached to these episodes and the uncertainty that she will come out unscathed (potentially a stroke) is very upsetting and worrying to our family and others who know and love her.

[redacted] is not what a young woman deserves when there is an alternative at the clinicians' fingertips, but which they are unable to dispense. For them, it must be like using a weak case of nails on a building that they know will eventually collapse. Frustrating I imagine, when they are in the business of saving lives.

For [redacted] family, it is nothing short of a constant state of anxiety. For [redacted] herself, the awful responsibility (through no fault of her own) of knowing this anxiety is put upon everyone who knows and loves her, on top of the constant fatigue and pressure of staying in full time employment is not an easy load to carry.

[redacted] has contributed very well to her country. She is well educated and would be a big loss to NZ if she pursued her only option to access the drug by moving to Australia. As a family, we find this a shameful option when 40 other countries value and look after their PNH sufferers by funding eculizumab.

I strongly urge PHARMAC to think again their decision to decline funding and to turn the lives of not only sufferers, but their families too, from a state of anxiety into a state of looking toward a positive future. How wonderful would that be for all!!

Thank you in anticipation

"Aunty" [redacted]

From: [redacted]
Sent: Wednesday, 19 June 2013 12:43 p.m.
To: eculizumabfeedback
Cc: OPP Review
Subject: Soliris for PNH patients
Attachments: PNH Pharmac.docx

To whom it may concern,

Please read attached letter in support of funding soliris for PNH patients.

Kind Regards

[redacted]

Subject: **Submission to PHARMAC's Proposal to decline a funding application for Soliris (eculizumab)**

To Whom it May Concern;

I support PNH patients in New Zealand in their fight to gain access to the life saving treatment Soliris.

I DO NOT support PHARMAC's proposal to decline funding for the Soliris treatment.

I support the PNH Support Association's proposition for Equity and Fairness which states:

i. **PHARMAC must return to the negotiating table** with the supplier of the Soliris treatment

ii. **PHARMAC must negotiate in good faith** toward funding Soliris for a minimum of 8 NZ PNH patients

iii. **PHARMAC must establish fair assessment criteria**, based on expert advice from the international haematological community, to assess patient need for the Soliris treatment

iv. **PHARMAC must amend its Operating Policies and Procedures** to acknowledge the right of rare disease patients to access life restoring and life saving treatments as in the specific example of the Soliris treatment

I have a brother living in [redacted] *with PNH and without the life saving treatment, he would not be here with us. Please think of these people as human beings with families and friends. Please re-consider your decision and fund soliris.*

Yours faithfully

[redacted]

From: mission to seafarers pt howard [redacted]
Sent: Wednesday, 31 July 2013 11:51 a.m.
To: eculizumabfeedback
Subject: Eculizumab

Southland Psoriasis Ass. Inc

[redacted]

[redacted]

[redacted]

To Sue Anne Yee
Therapeutic Group Manager
PHARMAC

Email: eculizumabfeedback@pharmac.govt.nz
Fax: 04 460 4995
Post: PO Box 10 254, Wellington 6143

Subject: **Submission to PHARAMC'S proposal to decline a funding application for Soliris (eculizumab)**

To whom it may concern:

I support the PNH patients in New Zealand in their fight to gain access to the lifesaving treatment Soliris.

I **DO NOT** support Pharmac's proposed intent to decline this treatment

- When Pharmac take this type of consultation to the public I should be able to rely on Pharmac to provide accurate and reliable information that will help guide responses from the community. In this consultation you have overstated the number of people affected with PNH which in turn over exaggerates the likely real cost of treatment. You did this back in 2011 when Pharmac staff reported to the Board that there would be up to 100 affected patients in New Zealand with Pompe disease. That treatment was declined, and it looks as though you intend to decline Soliris, again with dubious data as the basis for a decision. This is not acceptable and you should withdraw this consultation because of the misleading information in it, which is likely to skew responses from the public. .

- These patients have a right to life. Our health system has a duty to address their needs in a fair and equitable way. When Pharmac places so much emphasis on costs, cost-effectiveness and alternative use of the money, but does not address issues of rights, equity, fairness and community values, you are failing in your duty. The District Health Boards have this duty, and Pharmac is acting as their purchasing agency. You should use the same decision criteria and priorities that they have.

- Patients with rare diseases are disadvantaged because their condition is rare, and when treatments become available they are doubly affected by the high cost and the very small market due to small numbers. It is a denial of their right to health, and contrary to goals of "Equity of access, reducing inequalities and improving health outcomes for individuals and communities will guide our relationship and decision making", which are set out in the agreement between DHBs and Pharmac about how you decide things on their behalf.

- **Decision Criteria:** The decision criteria used to assess medicines are not fair for those who are affected by rare diseases. There should be an additional layer of decision-making for rare diseases that do not fit the standard cost effectiveness threshold for large populations. These additional layers already exist in Australia, England, Scotland and other places around the world, because they have recognised that it's a fair way to deal with the disadvantage of rare diseases.

- Pharmac's assumption that "best health outcomes" as mentioned in its legislation, can be strongly associated with calculation of Quality Adjusted Life Years, is too narrow a view of what is "best", especially when Pharmac's calculations do not take into account non-health-sector costs. The decision criteria should include a broader range of considerations that are important to patients, so that the decisions are made in a patient-centred way.

- Pharmac also has a responsibility for "health outcomes that are reasonably achievable". Your narrow perspective on technical assessment and budget management, to the exclusion of patient rights and interests from decisions, are an outcome that effectively discriminates against patients with rare diseases, and is not a reasonable outcome by any measure.

- Pharmac often emphasises the "tough decision" approach that some will be funded and others not. That is too simplistic and can also be unfair, as it is in this case. There are very good reasons to adopt an equitable approach that spreads medicine funding across as many areas of health need as possible so that no group is completely abandoned. That is the approach across a wide range of services in our health system and Pharmac should adopt a similar approach, rather than exclude and abandon certain groups.

- **I do not support** your intent to decline treatment for this group or other patients where there are therapies for rare diseases.

From: Stephanie Yee [redacted]
Sent: Tuesday, 30 July 2013 9:33 p.m.
To: eculizumabfeedback
Subject: submission for Soliris funding consultation

Sue Anne Yee
Therapeutic Group Manager
PHARMAC

Dear Sue and the Pharmac Board

Re: Submission opposing PHARMAC's Proposal to decline a funding application for Soliris (eculizumab)

As a patient advocate and health professional I strongly believe it would be inappropriate to flatly decline any funding of eculizumab for New Zealand patients with Paroxysmal Nocturnal Haemoglobinuria (PNH).

Soliris needs to be considered for funding under different decision criteria than currently employed because it is surrounded by unique circumstances with respect to the disease's rarity, the treatment being a maintenance therapy rather than curative, it's expense and the unavailability of alternative treatment options of comparable efficacy.

What is not questionable is the clinical efficacy of Soliris in significantly extending the lifetime and quality of life of a patient, particularly relevant in this case as PNH is diagnosed at a median age of 42 years.

In Pharmac's economic assessment more weighting should be given to the **medicine's effect on quality of life and the severity of the disease's impact on the patient's quality of life as well as the medicine's effect on the duration of life.**

While inarguably not being the most cost effective drug, at some point the value of a person's life comes down to more than cost effectiveness and must be measured and assessed using different formulae. While this is not my area of debate and I can understand there are limitations to the government's health budget, I ask Pharmac to consider listing Soliris on the Pharmaceutical Schedule under the following options:

a) Placing Soliris on the Hospital Medicines List (HML) with a 'Special Authority' with the criteria as discussed by the Pharmac haematology subcommittee of the patient having developed thrombosis despite anticoagulation or clone size >50% with systemic symptoms and evidence of active haemolysis.

b) Placing Soliris on the HML with a partial subsidising of total cost ex manufacturer.

c) If Soliris is not made available on the HML, the ability for a patient to apply for funding or partial funding under the NPPA scheme.

If PNH was a cancer, Soliris would in effect be the palliative treatment of choice without the need for several other interventions. Perhaps the answer lies in the need for a separate scheme for funding high-cost drugs as in the UK's Specialised Services programme. It just does not seem fair that someone who develops a condition through no fault of their own has their life hanging in the balance between a government agency and a drug company which manufactures the only efficacious treatment.

In summary,

I SUPPORT PNH patients in New Zealand in their fight to gain access to the lifesaving treatment Soliris.
I OPPOSE PHARMAC's proposal to decline funding for the Soliris treatment.
I support the PNH Support Association's proposition for Equity and Fairness which states:

1. **PHARMAC must return to the negotiating table** with the supplier of the Soliris treatment
2. **PHARMAC must negotiate in good faith** toward funding Soliris for a minimum of 8 NZ PNH patients
3. **PHARMAC must establish fair assessment criteria**, based on expert advice from the international haematological community, to assess patient need for the Soliris treatment
4. **PHARMAC must amend its Operating Policies and Procedures** to acknowledge the right of rare disease patients to access life restoring and lifesaving treatments as in the specific example of the Soliris treatment

Yours faithfully
Stephanie

Stephanie Yee
[redacted]

From: [redacted]
Sent: Wednesday, 31 July 2013 8:32 p.m.
To: eculizumabfeedback
Subject: submission to pharmac's proposal to decline a funding Soliaris

To Whom it May Concern;

I support PNH patients in New Zealand in their fight to gain access to the life saving treatment Soliris.

I DO NOT support PHARMAC's proposal to decline funding for the Soliris treatment.

I support the PNH Support Association's proposition for Equity and Fairness which states:

i.
PHARMAC must return to the negotiating table with the supplier of the Soliris treatment

ii.
PHARMAC must negotiate in good faith toward funding Soliris for a minimum of 8 NZ PNH patients

iii.
PHARMAC must establish fair assessment criteria, based on expert advice from the international haematological community, to assess patient need for the Soliris treatment

iv.
PHARMAC must amend its Operating Policies and Procedures to acknowledge the right of rare disease patients to access life restoring and life saving treatments as in the specific example of the Soliris treatment

(please include your personal statements here) This is very important to our family as the future of our grandson's father is in your hands.

Yours faithfully [redacted]

From: Sylvia Buarque Schiller Cooper [redacted]
Sent: Tuesday, 4 June 2013 8:20 a.m.
To: eculizumabfeedback; OPP Review
Subject: Letter of support to PNH patients in NZ

To Whom it May Concern;

I support PNH patients in New Zealand in their fight to gain access to the life saving treatment Soliris.

I DO NOT support PHARMAC's proposal to decline funding for the Soliris treatment.

I support the PNH Support Association's proposition for Equity and Fairness which states:

i. **PHARMAC must return to the negotiating table** with the supplier of the Soliris treatment

ii. **PHARMAC must negotiate in good faith** toward funding Soliris for a minimum of 8 NZ PNH patients

iii. **PHARMAC must establish fair assessment criteria**, based on expert advice from the international haematological community, to assess patient need for the Soliris treatment

iv. **PHARMAC must amend its Operating Policies and Procedures** to acknowledge the right of rare disease patients to access life restoring and life saving treatments as in the specific example of the Soliris treatment

We do not have much more to add apart from how would YOU feel if you know YOUR life could be saved but you had NO ACCESS to medicine?? It is an absurd that the lives of those people cannot be saved as we cant put a price tag on peoples' lives.

ACC covers people who are unwell from drinking and driving, the cost of self inflicted illness from smoking are also covered and we want to know why those people are denied treatment?

Families will suffer if they die and this does not need to happen. It is very sad that NZ is doing that to its people, heartbreaking really.

Yours faithfully

1 Sylvia Schiller-Cooper

2 Charles Brumpton Cooper

3 Tyler Schiller-Cooper

4 Regina Caiuby Schiller

From:	Tanz J [redacted]
Sent:	Thursday, 20 June 2013 12:31 p.m.
To:	eculizumabfeedback
Subject:	Submission to PHARMAC's Proposal to decline a funding application for Soliris (eculizumab)
Attachments:	proposition_equity_and_fairness_-_email_submission_template.doc

Please email *BOTH*

Subject: Submission to PHARMAC's Proposal to decline a funding application for Soliris (eculizumab)
Email: eculizumabfeedback@pharmac.govt.nz

AND

Subject: Submission in response to PHARMAC's consultation on Decision Critera.
Email: opp@pharmac.govt.nz

To Whom it May Concern;

I support PNH patients in New Zealand in their fight to gain access to the life saving treatment Soliris.

I DO NOT support PHARMAC's proposal to decline funding for the Soliris treatment.

I support the PNH Support Association's proposition for Equity and Fairness which states:

i. **PHARMAC must return to the negotiating table** with the supplier of the Soliris treatment

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iv. **PHARMAC must amend its Operating Policies and Procedures** to acknowledge the right of rare disease patients to access life restoring and life saving treatments as in the specific example of the Soliris treatment

Yours faithfully

Tania Jurgeleit

From: Tiffany Searle [redacted]
Sent: Tuesday, 4 June 2013 4:39 p.m.
To: eculizumabfeedback
Subject: 'Submission to PHARMAC's Proposal to decline a funding '

To Whom it May Concern;

I support PNH patients in New Zealand in their fight to gain access to the life saving treatment Soliris.

I DO NOT support PHARMAC's proposal to decline funding for the Soliris treatment.

I support the PNH Support Association's proposition for Equity and Fairness which states:

i. **PHARMAC must return to the negotiating table** with the supplier of the Soliris treatment

ii. **PHARMAC must negotiate in good faith** toward funding Soliris for a minimum of 8 NZ PNH patients

iii. **PHARMAC must establish fair assessment criteria,** based on expert advice from the international haematological community, to assess patient need for the Soliris treatment

iv. **PHARMAC must amend its Operating Policies and Procedures** to acknowledge the right of rare disease patients to access life restoring and life saving treatments as in the specific example of the Soliris treatment

Yours faithfully

Tiffany Searle

From: tilewa ojo [redacted]
Sent: Tuesday, 4 June 2013 8:57 p.m.
To: eculizumabfeedback
Subject: Help please

To Whom it May Concern;

I support PNH patients in New Zealand in their fight to gain access to the life saving treatment Soliris.

I DO NOT support PHARMAC's proposal to decline funding for the Soliris treatment.

I support the PNH Support Association's proposition for Equity and Fairness which states:

i. **PHARMAC must return to the negotiating table** with the supplier of the Soliris treatment

ii. **PHARMAC must negotiate in good faith toward funding Soliris for a minimum of 8 NZ PNH patients**

iii. **PHARMAC must establish fair assessment criteria,** based on expert advice from the international haematological community, to assess patient need for the Soliris treatment

iv. **PHARMAC must amend its Operating Policies and Procedures** to acknowledge the right of rare disease patients to access life restoring and life saving treatments as in the specific example of the Soliris treatment

Yours Faithfully

Miss Tilewa Ojo

From: Tim Seaholme [redacted]
Sent: Wednesday, 24 July 2013 6:52 p.m.
To: eculizumabfeedback
Subject: Submission to PHARMAC's Proposal to decline a funding application for Soliris (eculizumab)

To Whom it May Concern;
I support PNH patients in New Zealand in their fight to gain access to the life saving treatment Soliris.
I DO NOT support PHARMAC's proposal to decline funding for the Soliris treatment.
I support the PNH Support Association's proposition for Equity and Fairness which states:
PHARMAC must return to the negotiating table with the supplier of the Soliris treatment
PHARMAC must negotiate in good faith toward funding Soliris for a minimum of 8 NZ PNH patients
PHARMAC must establish fair assessment criteria, based on expert advice from the international haematological community, to assess patient need for the Soliris treatment
PHARMAC must amend its Operating Policies and Procedures to acknowledge the right of rare disease patients to access life restoring and life saving treatments as in the specific example of the Soliris treatment

Please treat this as you would if a family member was someone who this could make a real difference for.

Yours faithfully

Tim Seaholme

From: [redacted]
Sent: Sunday, 2 June 2013 3:10 p.m.
To: eculizumabfeedback
Subject: Submission to PHARMAC's Proposal to decline a funding application for Soliris (eculizumab)

To Whom it May Concern

I support PNH patients in New Zealand in their fight to gain access to the life saving treatment Soliris.

I DO NOT support PHARMAC's proposal to decline funding for the Soliris treatment.

I support the PNH Support Association's proposition for Equity and Fairness which states:

i. **PHARMAC must return to the negotiating table** with the supplier of the Soliris treatment

ii. **PHARMAC must negotiate in good faith** toward funding Soliris for a minimum of 8 NZ PNH patients

iii. **PHARMAC must establish fair assessment criteria**, based on expert advice from the international haematological community, to assess patient need for the Soliris treatment

iv. **PHARMAC must amend its Operating Policies and Procedures** to acknowledge the right of rare disease patients to access life restoring and life saving treatments as in the specific example of the Soliris treatment

I feel very strongly that Soliris be funded. I personally know a little boy who will benefit from Soliris treatment and it seems so unfair that this little boy's mother cannot afford to save her son's life.

Yours faithfully

[redacted]

From: [redacted]
Sent: Wednesday, 31 July 2013 5:49 p.m.
To: eculizumabfeedback
Subject: Funding for Soliris

My sister who lives in [redacted] has had to start treatment with Soliris and basically without the funding of this medicine in New Zealand she will never be able to return to her country of birth.

I urge that reconsideration be given to Pharmac's decision to disallow Soliris as a subsidised treatment for those with this awful affliction PNH. Surely there must be room to negotiate a satisfactory supply cost. The news of the NHS in England being exploited by drug companies makes grim reading and one would hope New Zealand does not allow itself to become a victim of such behaviour.Please make a stand.

Thank you.

[redacted].

From: Venus Flaws [redacted]
Sent: Tuesday, 4 June 2013 11:40 a.m.
To: eculizumabfeedback; OPP Review
Attachments: proposition_equity_and_fairness_-_email_submission_VFLAWS.doc

Please email *BOTH*

Subject: Submission to PHARMAC's Proposal to decline a funding application for Soliris (eculizumab)
Email: eculizumabfeedback@pharmac.govt.nz

AND

Subject: Submission in response to PHARMAC's consultation on Decision Critera.
Email: opp@pharmac.govt.nz

To Whom it May Concern;

I support PNH patients in New Zealand in their fight to gain access to the life saving treatment Soliris.

I DO NOT support PHARMAC's proposal to decline funding for the Soliris treatment.

I support the PNH Support Association's proposition for Equity and Fairness which states:

i. **PHARMAC must return to the negotiating table** with the supplier of the Soliris treatment

ii. **PHARMAC must negotiate in good faith** toward funding Soliris for a minimum of 8 NZ PNH patients

iii. **PHARMAC must establish fair assessment criteria**, based on expert advice from the international haematological community, to assess patient need for the Soliris treatment

iv. **PHARMAC must amend its Operating Policies and Procedures** to acknowledge the right of rare disease patients to access life restoring and life saving treatments as in the specific example of the Soliris treatment

(please include your personal statements here)

Yours faithfully

Venus Flaws

From: [redacted] on behalf of Vimal Patel [redacted]
Sent: Sunday, 2 June 2013 10:48 a.m.
To: eculizumabfeedback
Subject: Submission to PHARMAC's Proposal to decline a funding application for Soliris (eculizumab)

To Whom it May Concern;

I support PNH patients in New Zealand in their fight to gain access to the life saving treatment Soliris.

I DO NOT support PHARMAC's proposal to decline funding for the Soliris treatment.

I support the PNH Support Association's proposition for Equity and Fairness which states:

i. **PHARMAC must return to the negotiating table** with the supplier of the Soliris treatment

ii. **PHARMAC must negotiate in good faith** toward funding Soliris for a minimum of 8 NZ PNH patients

iii. **PHARMAC must establish fair assessment criteria**, based on expert advice from the international haematological community, to assess patient need for the Soliris treatment

iv. **PHARMAC must amend its Operating Policies and Procedures** to acknowledge the right of rare disease patients to access life restoring and life saving treatments as in the specific example of the Soliris treatment

Yours faithfully,

Vimal Patel

From: [redacted]
Sent: Tuesday, 30 July 2013 3:09 p.m.
To: eculizumabfeedback
Subject: Eculizumab Consultation Document
Attachments: PHARMAC Pharmac submission.doc

To Whom it May Concern: please see the attached submission regarding the above consultation document. Would you please acknowledge receipt of this message and the attached submission. Thanks

[redacted]

PHARMAC Proposal to Decline Fundng for the Treatment Eculizumab (SOLIRIS) for the Treatment of Paroxysmal Nocturnal Haemoglobinuria (PNH)

I refer to the PHARMAC consultation document of 21 May 2013.

1. I submit that the proposal to decline funding for SOLIRIS since it would:
   - represent a breach of the Human Rights Act 1993 which states, among other things, that *it is unlawful to deny, or treat a person less favourably, on any of the prohibited grounds of discrimination, including disability, race or ethnicity, sex, or age.*
   - be in contradiction of a stated objective of the New Zealand Public Health and Disability Act 2000 of *ensuring the best care and support of those in need of services.*
2. It would also be in breach of the **Universal Declaration of Human Rights** and the **International Covenant on Economic Social and Cultural Rights,** to which agreements New Zealand is a signatory.
3. I further submit that declining to fund SOLIRIS would be unjust and immoral since it would not only discriminate against a small number of sufferers from a rare disease, but would knowingly condemn them to living with a poor quality of life: frequent hospital visits, ongoing pain, loss of energy, and loss of income due to absence from work with frequent illness. It would also knowingly shorten the life span of these PNH sufferers.
4. The consultation document presents an exaggerated estimate of the cost of SOLIRIS. PHARMAC claims there are currently 12-20 patients who would be eligible for SOLIRIS treatment when the actual known number at this time is eight. Moreover, PHARMAC uses the 'list' cost of $600,000 as the annual cost per patient when PHARMAC knows the supplier is open to negotiating a lower cost.
5. The consultation document further claims that funding SOLIRIS would deprive 40,000 other patients of treatments that could be funded instead. This is a gross exaggeration. First, the number of PNH sufferers that would receive SOLIRIS is no more than eight. Secondly, it implies that there are no savings that could possibly be made elsewhere. Finally, it implies that PHARMAC would not succeed with making a case to the Government for additional funding needed to fund SOLIRIS. The question arises, therefore, has PHARMAC made a case requesting additional funding?
6. It is not clear from the document or from the economic outlines provided on the PHARMAC website, that the cost-effective analysis has taken full account of the

costs to the public health system of PNH sufferers not receiving SOLIRIS, which, as the case of my daughter indicates (Appendix 1), is considerable. Nor is it clear that the loss of productivity and income arising from the illness has been accounted for. These are all real costs to the Public Health System, to the individuals, and to the economy.

7. It is extraordinary that PHARMAC proposes to ignore the advice of its own professionals, the Haematology Subcommittee, which, based on its consideration of all the evidence, has recommended that SOLIRIS should be funded.

8. Equally extraordinary is the fact that PHARMAC proposed to decline the use of SOLIRIS when it is being provided to PNH sufferers in some 40 countries. This suggests that either PHARMAC possesses information regarding the efficacy of the drug, or these countries have information which it is the duty of PHARMAC to obtain. Perhaps it also implies that other countries take a more humane approach to assisting those suffering from rare diseases and disorders than PHARMAC. Or does it mean that other countries place a higher value of their citizens lives that PHARMAC has for New Zealand citizens.

9. I commend the experience of my daughter [redacted], a PNH sufferer currently receiving SOLIRIS courtesy of the supplier (see attachment). I suggest her story clearly indicates firstly, the considerable cost to the public health system prior to her receiving SOLIRIS; and secondly, the transformation in her quality of life since receiving this miracle drug. This transformation is no thanks to PHARMAC. It is the direct result of the dedication of the [redacted] Hospital Staff and to the compassionate generosity of Alexion, the supplier of SOLIRIS.

10. Should PHARMAC finally decide not to fund SOLIRIS, then I challenge the members of PTAC to personally face the PNH sufferers who would be directly disadvantaged and who are currently in desperate need of the relief that SOLIRIS would bring. I challenge you to invite these eight unfortunate people to Wellington, and tell them face-to-face: we are sorry you have this rare disease and we know there is a drug which would relieve your suffering, and we also know that the drug would extend your life expectancy, but we regret to tell you that we don't consider you are worth the cost! For that is exactly what PHARMAC is proposing: these lives are not worth saving!

Attachment: Patient History: [redacted]

[redacted]

[redacted]

[redacted]

[redacted]

[redacted]

[redacted]

[redacted]

[redacted]

[redacted]

[redacted]

[redacted]

[redacted]

[redacted]

[redacted]

From: Warwick.Trish Darrow [redacted]
Sent: Tuesday, 18 June 2013 8:41 a.m.
To: eculizumabfeedback
Subject: fund solirus

To whom it may concern,
We have read about this dreadful blood disease PNH which effects young and old.
We believe Pharmac should fund Solirus and not take into there own hands being Judge and Jury letting sick people die.
Pharmac should join hands with our Australian country cousins and a large majority of other countries who already fund Solirus.
Please give these sick people a chance for a life so they can live like others.
Yours Faithfully,
Warwick Darrow
Patricia Darrow

From: [redacted]
Sent: Monday, 22 July 2013 4:09 p.m.
To: eculizumabfeedback
Subject: PNH

RE: **Submission opposing PHARMAC'S Proposal to decline a funding application for Soliris (eculizumab)**

To Whom it May Concern;

I SUPPORT PNH patients in New Zealand in their fight to gain access to the lifesaving treatment Soliris.

I OPPOSE PHARMAC's proposal to decline funding for the Soliris treatment.

I support the PNH Support Association's proposition for Equity and Fairness which states:

1. **PHARMAC must return to the negotiating table** with the supplier of the Soliris treatment
2. **PHARMAC must negotiate in good faith** toward funding Soliris for a minimum of 8 NZ PNH patients
3. **PHARMAC must establish fair assessment criteria**, based on expert advice from the international haematological community, to assess patient need for the Soliris treatment
4. **PHARMAC must amend its Operating Policies and Procedures** to acknowledge the right of rare disease patients to access life restoring and lifesaving treatments as in the specific example of the Soliris treatment

Our daughter and Step-daughter is one of these people that need this treatment, what if it was one of yours???????????????

Yours faithfully

[redacted]

From: Weihana Delamere [redacted]
Sent: Tuesday, 4 June 2013 11:07 a.m.
To: eculizumabfeedback

Sue Anne Yee
Therapeutic Group Manager
PHARMAC

Re: Proposal to decline a funding application for eculizumab

I would like to submit comment on the proposed refusal to fund this life-saving medicine. Whilst the concerns over its cost are understandable the statement "There are always more medicine funding applications than the available budget will allow" is troubling because it suggests that Pharmac is lumping all medicine as being the same whereas the public perception (or at least my perception) is that this is a more extreme case where the funding may be considerable but so is the benefit to the individual patient. While you mention 40000 other patients missing out are all these patients under the same circumstances? To view utility as merely a numbers game seems rather callous (no offense).

You state:

"Even if DHBs had much more money available, at the current price, eculizumab is not cost-effective and would be likely to be at the back of the queue of medicines that could be funded."

But how can you say it is not cost effective when it appears that we are talking life and death here. Have you worked out the value of someone's life? And why is it your job to say how much more money should be made available? It's easy to understand that with a limited budget Pharmac must be in the business of making trade-offs when it comes to funding decisions. But it should be the job of the public to decide how important they think life saving medicine is compared to other priorities. Put the information out there for the public to judge. We spend so much money on other things that the public should be able to decide whether to spend more on life saving medicine or to put interest on student loans, or to raise taxes, or to stop wasting money on a pointless military that could defeat no one. Why should these decisions be the domain of a few bureaucrats with a defeatist attitude?

Bottom line is that if there is treatment that is life saving and we do not try it then we as a nation need to re-evaluate our priorities. The notion that we just can't afford it doesn't wash. We spend so much on things which are not life and death. If trade-offs are to be made they can be made in other areas. Or at least give the public the information they need to make that decision. People shouldn't die just so Bill English can have his budget or so the public doesn't have to properly evaluate the value of the handouts they may vote themselves. Do I love an interest free student loan? Absolutely. It's a great opportunity and limits the cost of my education. But do I think I should get it at the expense of someone who has to go without life saving medicine? Absolutely NOT! But we do not even get to have this discussion because all you want to do is throw your hands in the air and say "nothing we can do".

I have yet to meet someone who doesn't think this drug should be funded. This from people all over the political spectrum. Instead of marketing for a government that doesn't want to rock the budgetary boat, how about you get in behind the public who wants to make this work and to FIND A WAY to fund this medicine?

Yours sincerely,

Weihana Delamere
E-mail [redacted]
[redacted]

From: [redacted]
Sent: Tuesday, 30 July 2013 11:19 p.m.
To: eculizumabfeedback
Subject: Proposal to Decline Funding for Eculizumab
Attachments: Mum's submission.docx

Please find attached a submission re the above.

Regards
[redacted]

SUBMISSION ABOUT PHARMAC'S PROPOSAL TO DECLINE FUNDING FOR THE DRUG ECULIZUMAB (SOLIRIS) FOR THE TREATMENT OF PAROXYSMAL NOCTURNAL HEMOGLOBUNARIA - PNH

We are [redacted], parents of PNH sufferer [redacted].

Our first concern is for the future health of our daughter and what PHARMAC's proposal to decline funding for the drug eculizumab (soliris) means for her and for our family.

Only one word comes to mind….. devastation …….but we'll come back to this.

Our second concern is for the disappointment we feel about the inaccuracies in PHARMAC's consultation document, not something we expected from an organisation charged with making decisions that literally determine the every day quality of life for many New Zealanders.

PHARMAC has a huge responsibility, and we have the right to expect they have a decision making process that is robust and trustworthy. We should be utterly confident that decisions are based on the most up-to-date and accurate information available to them at the time. Unfortunately, this has not been the case.

We also expected transparency during the decision-making process. Lack of transparency immediately rings alarm bells, and alarm bells have been ringing. This has been frustrating and unacceptable.

We expect PHARMAC would argue that they are delivering their legislative objective which is "to obtain the best health outcomes that are reasonably achievable within the budget".

It's all down to interpretation of course. Our family's interpretation is that PHARMAC have fallen short. Given we don't see anywhere, the words "excluding those people with rare diseases", we assumed all New Zealanders' needs would be debated fairly and honestly.

However, the needs of people with the rare disease PNH, have been unfairly debated, with the outcome being a proposal to decline access to the life changing drug eculizumab.. This can only be interpreted as discrimination.

If PHARMAC's budget is not adequate, which seems to be the catch cry, then it is their responsibility to address the issue, put wheels in motion to make change.

Ironically eculizumab is available in 40 other countries – I'm sure some of them would be very happy to share their policies, processes etc with New Zealand from which we can surely incept our own model. It is understandable an increased budget might be required, so maybe central government needs to be prompted here.

Now back to what PHARMAC's proposal to decline funding for eculizumab means for [redacted] and our family.

We know making emotional pleas to PHARMAC has no impact when it comes to the allocation of funds. However, as a family watching [redacted] battle with PNH, it is impossible not to do just that – this is our emotional plea.

We are constantly aware that [redacted] health is precarious and know only too well the battle she fights every day. We won't list the physical problems she endures, it would take too long. We won't list the constant medical check-ups, blood tests, etc she has to endure, you would get bored. [redacted] we won't talk about the fact the drug is available in other 40 countries, New Zealand is where she has was born.

All loving parents want only good things for their children – we want a miracle for [redacted]. Impossible you might say, but talk to a PNH sufferer lucky enough to have access to Soliris and see if they don't agree…… a miracle is possible.

Eculizumab has proven efficacy in terms of changing the lives of PNH sufferers, it gives them a normal life expectancy, they can live a seemingly disease free life.

Put yourself in our position

[redacted]

[redacted]

From: Wendy Butler [redacted]
Sent: Saturday, 1 June 2013 3:41 p.m.
To: eculizumabfeedback; OPP Review
Attachments: proposition_equity_and_fairness_-_email_submission_template.doc

To Whom it May Concern;

I support PNH patients in New Zealand in their fight to gain access to the life saving treatment Soliris.

I DO NOT support PHARMAC's proposal to decline funding for the Soliris treatment.

I support the PNH Support Association's proposition for Equity and Fairness which states:

i. **PHARMAC must return to the negotiating table** with the supplier of the Soliris treatment

ii. **PHARMAC must negotiate in good faith** toward funding Soliris for a minimum of 8 NZ PNH patients

iii. **PHARMAC must establish fair assessment criteria**, based on expert advice from the international haematological community, to assess patient need for the Soliris treatment

iv. **PHARMAC must amend its Operating Policies and Procedures** to acknowledge the right of rare disease patients to access life restoring and life saving treatments as in the specific example of the Soliris treatment

Yours faithfully

Wendy Butler.

To Whom it May Concern;

I support PNH patients in New Zealand in their fight to gain access to the life saving treatment Soliris.

I DO NOT support PHARMAC's proposal to decline funding for the Soliris treatment.

I support the PNH Support Association's proposition for Equity and Fairness which states:

i. **PHARMAC must return to the negotiating table** with the supplier of the Soliris treatment

ii. **PHARMAC must negotiate in good faith** toward funding Soliris for a minimum of 8 NZ PNH patients

iii. **PHARMAC must establish fair assessment criteria,** based on expert advice from the international haematological community, to assess patient need for the Soliris treatment

iv. **PHARMAC must amend its Operating Policies and Procedures** to acknowledge the right of rare disease patients to access life restoring and life saving treatments as in the specific example of the Soliris treatment

Yours faithfully

Wendy Butler.

From: Xin Cheng [redacted]
Sent: Monday, 3 June 2013 3:20 p.m.
To: eculizumabfeedback
Subject: Submission to PHARMAC's Proposal to decline a funding application for Soliris (eculizumab)

To Whom it May Concern,

I support PNH patients in New Zealand in their fight to gain access to the life saving treatment Soliris.

I DO NOT support PHARMAC's proposal to decline funding for the Soliris treatment.

I support the PNH Support Association's proposition for Equity and Fairness which states:

i. PHARMAC must return to the negotiating table with the supplier of the Soliris treatment

ii. PHARMAC must negotiate in good faith toward funding Soliris for a minimum of 8 NZ PNH patients

iii. PHARMAC must establish fair assessment criteria, based on expert advice from the international haematological community, to assess patient need for the Soliris treatment

iv. PHARMAC must amend itsOperating Policies and Procedures to acknowledge the right of rare disease patients to access life restoring and life saving treatments as in the specific example of the Soliris treatment

I find it very bizarre that citizens of countries like Turkey and Brazil have access to this treatment, yet New Zealanders being deprived of this life-saving treatment.

Yours faithfully

Xin Cheng

From: [redacted]
Sent: Sunday, 2 June 2013 12:17 a.m.
To: eculizumabfeedback
Subject: Proposal to Decline Funding for Soliris

To Whom it May Concern;

I support PNH patients in New Zealand in their fight to gain access to the life saving treatment Soliris.

I DO NOT support PHARMAC's proposal to decline funding for the Soliris treatment.

I support the PNH Support Association's proposition for Equity and Fairness which states:

i. **PHARMAC must return to the negotiating table** with the supplier of the Soliris treatment

ii. **PHARMAC must negotiate in good faith** toward funding Soliris for a minimum of 8 NZ PNH patients

iii. **PHARMAC must establish fair assessment criteria**, based on expert advice from the international haematological community, to assess patient need for the Soliris treatment

iv. **PHARMAC must amend its Operating Policies and Procedures** to acknowledge the right of rare disease patients to access life restoring and life saving treatments as in the specific example of the Soliris treatment

Last year [redacted] passed away while getting a bone marrow transplant. [redacted] I also petitioned for Soliris to be funded in Australia, and it was accepted. [redacted] Please, if you could change your mind, I know how much it would mean to PNH suffers and their family and friends alike.

Yours faithfully,

[redacted]

From: [redacted]
Sent: Thursday, 27 June 2013 1:57 p.m.
To: eculizumabfeedback
Subject: Submission to PHARMAC's Proposal to decline a funding application for Soliris (eculizumab)

To Whom it May Concern;

I support PNH patients in New Zealand in their fight to gain access to the life saving treatment Soliris.

I DO NOT support PHARMAC's proposal to decline funding for the Soliris treatment.

I support the PNH Support Association's proposition for Equity and Fairness which states:

i. **PHARMAC must return to the negotiating table** with the supplier of the Soliris treatment

ii. **PHARMAC must negotiate in good faith** toward funding Soliris for a minimum of 8 NZ PNH patients

iii. **PHARMAC must establish fair assessment criteria**, based on expert advice from the international haematological community, to assess patient need for the Soliris treatment

iv. **PHARMAC must amend its Operating Policies and Procedures** to acknowledge the right of rare disease patients to access life restoring and life saving treatments as in the specific example of the Soliris treatment

As a PNH sufferer in [redacted] *with access to Soliris when I need it, I have peace of mind that my quality of life will be drastically improved and my life expectancy will be extended due to this life saving drug. It is unfair that rare diseases are neglected and don't get the attention, funding and charity support etc of more widespread illnesses. When you are a sufferer and someone who loves a sufferer this makes no sense at all. Having such a disease is not just about the physical but also the mental suffering, not just about the patient but about the patient's loved ones. Please support these sufferers as has been done in so many other countries.*

Everyone's life is worth saving.

Yours faithfully

[redacted]

From: [redacted]
Sent: Tuesday, 30 July 2013 8:01 p.m.
To: eculizumabfeedback; OPP Review
Subject: Submission opposing PHARMAC's Proposal to decline a funding application for Soliris (eculizumab)

To Whom it May Concern;

I SUPPORT PNH patients in New Zealand in their fight to gain access to the lifesaving treatment Soliris.

I OPPOSE PHARMAC's proposal to decline funding for the Soliris treatment.

I support the PNH Support Association's proposition for Equity and Fairness which states:

i. **PHARMAC must return to the negotiating table** with the supplier of the Soliris treatment

ii. **PHARMAC must negotiate in good faith** toward funding Soliris for a minimum of 8 NZ PNH patients

iii. **PHARMAC must establish fair assessment criteria,** based on expert advice from the international haematological community, to assess patient need for the Soliris treatment

iv. **PHARMAC must amend its Operating Policies and Procedures** to acknowledge the right of rare disease patients to access life restoring and lifesaving treatments as in the specific example of the Soliris treatment

I am a [redacted] year old [redacted] living with Paroxysmal Nocturnal Hemoglobinuria (PNH). I was diagnosed with PNH in [redacted] after experiencing severe abdominal pain, chronic fatigue and a clot in my lung.

At the time Soliris was not available, so for the following [redacted] years of my life my family and I searched for some "cure" to PNH. As we found out the only "cure" for PNH is a bone marrow transplant. This was not a viable option for me as I did not have a match.

[redacted]

As you will know PNH is a debilitating chronic illness that affects your everyday life. Even having a shower each day is an effort, let alone trying to work, and even enjoy social activities.

Now with Soliris I am able to live a "normal life" - I have been able to work full-time, go to the gym and participate in social activities. I now feel that I am a functioning member of society, and feel that I am actually living life, rather than watching it pass by.

I strongly believe that Soliris should be available to other PNH sufferers. It is their <u>only</u> chance at living a "normal life". Please consider the impact that Soliris will have on someone with PNH, so they can have a chance at enjoying a "normal life" like me.

Yours faithfully

[redacted]

From: [redacted]
Sent: Tuesday, 30 July 2013 9:13 p.m.
To: eculizumabfeedback
Subject: eculizumab feedback from [redacted]
Attachments: [redacted]

Please find feedback attached

Submission to Pharmac regarding funding for Soliris treatment

[redacted]

As a sufferer of PNH for the past [redacted] years I have lived in hope that one day modern medicine will provide me with the medication required to combat this condition.

I have always tried to remain positive in my approach to life despite the limitations that living with PNH has put before me. I have raised [redacted] children and have worked alongside my husband to build up a successful [redacted] business. During the last [redacted] years it has become blatantly clear that the treatments I receive at present being – prednisone, folic acid and warfarin together with approximately three blood transfusions per annum – are not now enough to ensure a sustainable quality of life.

I was absolutely ecstatic when I first heard of Soliris – the miracle breakthrough I had been waiting for, only to be devastated when it was revealed that due to price I would not be able to receive it. I felt let down by Pharmac who has the power to say whether I should receive this treatment or not. Therefore I strongly encourage Pharmac to reconsider their decision not to fund Soliris and give those of us who, through no fault of our own have been cursed with this condition, a chance to live a 'normal' life.

Please remember no price should be put on a life – no matter the cost.

From: [redacted]
Sent: Wednesday, 31 July 2013 12:21 p.m.
To: eculizumabfeedback
Subject: Proposal To Decline Funding - Soliris/Eculizumab

Dear Pharmac,

Thank you for considering the funding of Eculizumab within the new Zealand market. In the interests of fairness I very much appreciate that this process is to some extent open and that your proposal to decline funding is open for submissions and feedback.

This process is of course very emotive for patients and their families and I am no exception – as far as I can determine my [redacted] within New Zealand presently diagnosed with PNH. [redacted] was diagnosed with PNH in [redacted] At this point in the development of her disease she has remained free of blood clots and for this we are very grateful. Even if Eculizumab was presently funded within New Zealand I would not expect that [redacted] would be at the stage in [redacted] disease where such treatment would be necessary – I believe that Eculizumab would be/should be only for those with the most advanced symptoms and high risk of blood clotting in particular.

Your recommendation to not fund Eculizumab does mean that the future looks much more bleak for [redacted] than it would if this treatment was available, and this is of course very concerning. I note that you suggest that there are 60-70 PNH patients within NZ, and that 12-20 patients may "qualify" for Eculizumab if it was available. With respect these are not numbers that my research and knowledge of the PNH community support – as far as I can ascertain there are perhaps 25 sufferers of PNH within New Zealand and therefore with restricted qualifying criteria Eculizumab would cover a much smaller number of patients – perhaps 8-10 at the more advance stages of the disease where this drug provides the most benefit.

I have read the papers you have made available on your website and I conclude 2 things –

1) The Eculizumab is indeed effective at treating the symptoms of PNH, and this is supported by clinical evidence and a recommendation by PTAC, and whilst I recognise that there are other therapies that can assist with the managing the symptoms none of these treatments work as effectively to manage this very rare disease.
2) The cost/benefit ratio does not stack up against other treatments and/or other rare disease treatments.

I believe that the outrageous cost of this effective and proven treatment to be responsible for the funding dilemma we find ourselves in.

However may I ask a few questions?

1) Your papers state that you support the drug Idursulfase (via NPPA) at a cost of $419,000 per year. Does this indicate the price point, below which you would (re)consider funding Eculizumab?
2) Has Pharmac attempted to seriously negotiate with Alexion? I understand that Alexion may well have made a "discounted offer" to you however this is merely a starting point to work downwards from.
3) Has Pharmac indicated to Alexion the price point at which they would be prepared to buy? I know the present asking price is extremely high, however Alexion would no doubt be willing to negotiate. I understand that Pharmac may consider that the amount of discount in both percentage and absolute terms that would be required to meet cost/benefit ratios to then be commercially unviable for Alexion, but given the sunk development costs for Soliris it's certainly worthy of discussion with them.

4) Would Pharmac consider a part funding model where perhaps a DHB, another govt dept or NGO shared in the cost?
5) When does Soliris come off patent?
6) Soliris is starting to make its presence felt in the treatment of other rare diseases, so will Pharmac then consider each disease separately or consider the patient group as a whole? I would hope (naively perhaps) that if the patient numbers increased as Soliris is proven effective against other diseases that its asking price may fall.

Whilst I appreciate that the main reason for the proposal to decline this funding is based on cost, and as stated above I understand why this would be the case, my feeling is that there should be much more focus on negotiation with Alexion with the aim to come to an acceptable financial agreement before the final decision to decline this funding is made by you.

Thanks for taking the time to read to this point. As stated I understand your reasons over the cost of Soliris however at the same time [redacted] and other sufferers will in the future be directly affected by any decision that you make. Rather than arguing the cost is too high (we both agree on that) I feel that this could progress to the benefit of those affected by PNH, Pharmac and Alexion if the cost could be brought down to a more commercially viable level. I believe this could be achievable too if negotiations between the parties could be undertaken in an effort to provide a very real solution for this very serious and debilitating disease.

Yours faithfully

[redacted]

Please email *BOTH*

Subject: Submission to PHARMAC's Proposal to decline a funding application for Soliris (eculizumab)
Email: eculizumabfeedback@pharmac.govt.nz

AND

Subject: Submission in response to PHARMAC's consultation on Decision Critera.
Email: opp@pharmac.govt.nz

To Whom it May Concern;

I support PNH patients in New Zealand in their fight to gain access to the life saving treatment Soliris.

I DO NOT support PHARMAC's proposal to decline funding for the Soliris treatment.

I support the PNH Support Association's proposition for Equity and Fairness which states:

i. **PHARMAC must return to the negotiating table** with the supplier of the Soliris treatment

ii. **PHARMAC must negotiate in good faith** toward funding Soliris for a minimum of 8 NZ PNH patients

iii. **PHARMAC must establish fair assessment criteria**, based on expert advice from the international haematological community, to assess patient need for the Soliris treatment

iv. **PHARMAC must amend its Operating Policies and Procedures** to acknowledge the right of rare disease patients to access life restoring and life saving treatments as in the specific example of the Soliris treatment

Having AA PNH myself..it's impossible to understand why "not providing" whatever medical help you can to the PNH patients of NZ would be a choice you would make. PHARMAC has the option in "III" to discriminate by fair assessment criteria. Soliris is not a treatment for all but thankfully it is for some.

As a non-soliris PNH patient of [redacted] *I urge you to give them that chance.*

Yours faithfully

[redacted]

(SAAPNH patient)

[redacted]

From: [redacted]
Sent: Monday, 3 June 2013 3:54 p.m.
To: eculizumabfeedback; OPP Review
Subject: proposition_equity_and_fairness_-_email_submission_template.doc
Attachments: proposition_equity_and_fairness_-_email_submission_template.doc

[redacted]

[redacted]

[redacted]

[redacted]

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As a non-soliris PNH patient of [redacted] *I urge you to give them that chance.*

Yours faithfully

[redacted]

(SAAPNH patient)

[redacted]

From: [redacted]
Sent: Wednesday, 12 June 2013 3:50 a.m.
To: eculizumabfeedback
Subject: Submission to PHARMAC's Proposal to decline a funding application for Soliris (eculizumab)

To whom it may Concern,

I am a New Zealander and I suffer from PNH. I am [redacted] years old and I was diagnosed in Auckland [redacted]

[redacted] has recently offered me treatment with Soliris due to the continuing seriousness of my condition. But should Soliris continue not to be funded in New Zealand, I, my New Zealand-born partner and any future children we may have, will effectively be exiled from our homeland, as once I embark on the drug treatment, I must remain on it or face life threatening consequences.

I will therefore be forced to choose my physical health over my emotional health which would derive from being able to return to New Zealand to live, and be surrounded by family, in the country in which I grew up and spent much of my adult life.

This is an unacceptable position in which to be placed. I have delayed starting treatment with Soliris (possibly to my detriment) because I am waiting for the outcome of the current PHARMAC consultation regarding this issue. If Soliris is not funded in New Zealand, this will affect whether I commence this treatment which I anticipate will provide me with an increased quality of life and also an increased life expectancy.

Since arriving in [redacted], I have been provided with exemplary care [redacted] as a dedicated PNH nurse who organises a PNH patient support group and is available by email and telephone at all times. She also makes arrangements for the treatment of patients receiving Soliris.

[redacted]

Notwithstanding that I do my best to manage my continuing symptoms, my PNH clone (which is 80%) has not reduced and my symptoms are becoming more challenging to tolerate.

However, the most relevant consideration is the ever present risk of thrombosis which I am aware could occur at any time with serious and perhaps fatal consequences.

[redacted]

[redacted]

[redacted]

[redacted]

The decision whether to follow my doctors' advice and begin Soliris treatment is not an easy one for a number of reasons including the requirement to have bi-weekly infusions which I will need to accommodate into my already full work schedule.

Most importantly I am of course aware that Soliris is not currently funded in New Zealand. As I am sure you will be aware, once treatment with Soliris is commenced, it cannot be stopped without elevating the inherent risks of PNH including the increased risk of thrombosis.

Therefore the fact that Soliris treatment is not funded in New Zealand is perhaps the most significant factor in my decision whether to commence this recommended treatment. I always intended to return to New Zealand to live. Should Soliris continue not to be funded in New Zealand, I will be prevented from returning to my homeland.

The personal consequences of not being able to live in New Zealand in the future are too numerous to list here. [redacted] also have a large extended family living in New Zealand who I will be prevented from having any real future contact with, not to mention close friends. My boyfriend with whom I live is also a New Zealander and should I be prevented from returning to the land of my birth, so too will he if we wish to be able to reside in the same country.

Additionally, any children I am lucky enough to be able to have (I understand that pregnancy is only possible for PNH sufferers who are undergoing Soliris treatment) will therefore be prevented from living in New Zealand, knowing any of their grandparents or other family members.

Apart from the important personal consequences this presents, from a professional perspective, I had also hoped to utilise the wealth of knowledge and experience I have gained while working in [redacted], on my return to New Zealand.

I urge you to reconsider your stance on the funding of this drug. I can't imagine how stressful it must be for the other New Zealanders with PNH and their families who are having to deal with this illness, knowing that there is a drug which could increase their quality of life and also their life expectancy but not having access to it.

I support PNH patients in New Zealand in their fight to gain access to the life saving treatment Soliris.

I DO NOT support PHARMAC's proposal to decline funding for the Soliris treatment.
I support the PNH Support Association's proposition for Equity and Fairness which states:

PHARMAC must return to the negotiating table with the supplier of the Soliris treatment

PHARMAC must negotiate in good faith toward funding Soliris for a minimum of 8 NZ PNH patients

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PHARMAC must amend its Operating Policies and Procedures to acknowledge the right of rare disease patients to access life restoring and life saving treatments as in the specific example of the Soliris treatment

I would be happy to provide further information should this be required.

Yours faithfully

[redacted]