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.