



21345 Miles Drive
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 **A**wareness, influencing & funding **R**esearch, and expanding **E**ducation 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 world 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,

A handwritten signature in black ink, appearing to read "Dean Suhr". The signature is fluid and cursive, with a large initial "D" and "S".

Dean Suhr, President
MLD Foundation