Summary of the proposal from Lysosomal Diseases NZ to establish a separate funding process for medicines that treat some rare disorders

Context

Mr John Forman, a rare disease advocate and Chair of Lysosomal Diseases NZ has expressed concern about the current processes for funding medicines for rare disorders and has proposed that there be a process for funding some of these medicines, partly separated from the PHARMAC process. He considers that these medicines are not funded because of cost concerns and that, in turn, there is a weakness in the way the funding system works. That is, he is concerned that it lacks 'equity' and excludes some patient groups. The Minister of Health has asked the Ministry to work with Mr Forman to explore the feasibility of the ideas. The purpose of this note is to describe Mr Forman's proposal for the purposes of ensuring the Ministry's analysis is informed by a clear understanding of it.

The proposal would be a significant shift from the current approach for determining access to publicly funded pharmaceuticals[1] and there are important policy and implementation issues that need consideration. This note does not attempt to go into these matters.

The proposal

The proposal is to establish a process to determine individual eligibility to access those publicly-funded pharmaceuticals contained in a defined list. It borrows elements from the Pharmaceutical Benefits Scheme and the Life-Saving Drugs Programme in Australia. The key elements of the proposal are set out below:

a) Medicines: Mr Forman's initial suggested list of medicines is set out in table one. These medicines are for the treatment of some rare disorders including lysosomal storage disorders. The diseases treated are genetic/metabolic conditions and the treatments are expensive. The initial suggested list in Table 1 are those that are on the Australian LSDP or PBS but declined for funding by Pharmac[2].or had a recommendation for decline from PTAC. This is proposed as the criteria for medicines to be included on the list.
b) Eligible patients: the proposal provides that all patients with a relevant condition would potentially be eligible. Additional criteria are likely to be needed depending on disease characteristics and available funding.
c) Decision maker: the proposal is that there be a committee[3] established to:
    i. Determine which medicines/disorders should be on the list
    ii. Identify patients with each of the diseases in question
    iii. Confirm an individual's eligibility for access and review this periodically
    iv. Decide of all eligible individuals who is 'most in need' and therefore should have priority access to treatment. Criteria would need to be developed and could include lack of access to any effective treatment for their condition, and assessment of their

[1] At present PHARMAC determines access for the New Zealand population and the Pharmaceutical Schedule contains the list of publicly-funded pharmaceuticals. It also considers individual access to medicines not on the Pharmaceutical Schedule in exceptional circumstances through the Named Patient Pharmaceutical Access programme (known as NPPA).

[2] PHARMAC is yet to confirm the status of these medicines.

[3] The High Cost Treatment Pool process has been suggested as potentially useful in this regard.

clinical circumstances, as factors used in setting priority. The committee would aim to spread the available funds across all these diseases and to treat the highest priority patients for each disease.

v. Determine on an ongoing basis whether new medicines should be added to, or removed from, the list

vi. Provide information on likely future treatment needs and priorities for patients with these diseases, to assist planning and budget setting

d) Funding: it is proposed that funding from the Pharmaceutical Budget be set aside to fund these medicines and that it be ring-fenced from being used for other purposes. Initially $5m per year has been discussed ($20m/four years) (acknowledging that additional funding could be used if it were made available). Note – the funding proposed is based on statements made by the Minister about what will be available. The prioritisation process is suggested only in the event that the funding is inadequate to meet the treatment needs of patients with these diseases.

e) Procurement: it is proposed that PHARMAC be tasked with procuring the medicines using, as far as it is able, its experience in commercial pharmaceutical negotiation to leverage the best price. Note – this provision is included because the Minister stated he wanted Pharmac to be involved. The preference of patient groups is that the scheme should reflect the earlier political promises and remove all responsibility for this scheme to the separate committee.

Table 1: Medicines proposed for Round 1 of the alternative process [Indicative]	
Agalsidase alfa (Replagal) and beta (Fabrazyme) for Fabry disease	
Alglucosidase alfa (Myozyme) for Pompe disease	
Eculizumab (Soliris) for PHN and aHUS	
Elosulfase alfa (Vizimin) for Morquio disease	
Idursulfase (Eleprase) for Hunter disease	
Ivacaftor (Kalydeco) for mutation-specific cystic fibrosis	
Laronidase (Aldurazyme) for Hurler disease	
Nitisinone (Orfadin) for Tyrosinemia Type 1	
Sapropterin dihydrochloride (Kuvan) for PKU	

Background to the proposal, prepared by Mr Forman

Access to "orphan" drugs for rare diseases has been a contentious issue for many years. Numerous discussion documents and reports have been prepared since 2005. In 2014 Pharmac set new policy and sought proposals under a $5 million pilot scheme to improve the number of proposals received for funding of these medicines.

The 2014 RFP indicated a number of diseases that appeared to be likely candidates for consideration under the pilot. Mr Forman maintains that of a then estimated 120 patients with these diseases, fewer than 5 would likely be treated now by approvals made from the pilot. He noted that several approvals under the pilot were in fact shifts of funds from exceptional circumstances (NPPA) to the schedule, and so not new funding for medicines not previously funded. Numbers in need of available treatments that are not funded, will be higher now – estimated about 150. The obvious implication is that policy to date has not made a significant impact and is unlikely to in the future unless there are changes, leaving a growing group of patients abandoned without treatment.

The tension in this debate is between a focus on equity, and a focus on health outcomes from a limited budget. Pharmac's policy and decisions have consistently maintained a strong focus on health outcomes as measured by QALY gains and alternative investments with available funds. This is consistent with their interpretation of their statutory mandate. In contrast, John refers to a variety of publications on universal healthcare, the sustainable development goals, distributive justice in health, the NZ H&D Act, and the NZ health strategy, as the basis for an equity approach that deals more adequately for those who tend to miss out in the current policy settings.

Leading up to both the 2014 and 2017 general elections, policy positions from the three parties now forming the government, all stated an intention to develop a separate fund for orphan drugs, to manage that away from Pharmac, and to have consumer involvement in decisions. The 2017 confidence and supply agreement did not include such a decision. But the Minister has stated his commitment to progress in funding treatments for rare diseases and his agenda for this is equity. (Radio NZ interview with Dr Collette Bromhead, CE of NZORD 21 Feb 2018).

A meeting between Mr Forman and the Minister on 3 April 2018 resulted in the outline of a proposal being put to the Minister on how a fairer system could be developed for funding of orphan drugs, and equity achieved. The Minister reinforced his concern for the plight of these patients and stated his intention that equity needs to be more specifically addressed in funding decisions. He asked the Ministry to work with Mr Forman to investigate the practicalities of how such a system could work.

Other considerations.

In initial conversations, the Ministry has discussed several related matters with Mr Forman and his responses are:

- The focus on rare genetic/metabolic diseases follows the Australian policy which specifically targets a group of diseases and their treatments that regularly fail standard cost-effectiveness evaluations in the PBAC and get referred to their LSDP.

- Rare cancers and other diseases where small subsets of patients might raise similar issues, may need separate consideration and a similar scheme, though they could be considered under this scheme if the budget allocation was adequate to deal with them too.
- Consumer engagement in this process is occurring now in the design of it, and would ideally continue in the special committee which determines funded access. This will be consistent with commitments frequently made by the 3 parties in prior election policies.
- For Pharmac to manage the whole process under their recently announced RFP for medicines for rare diseases (a follow up to their earlier pilot), there would need to be a significant change of focus on their part regarding equity. In the past they have been very determined in rejecting any special consideration of equity.
- Though Pharmac's new factors for consideration can include equity, they give no indication that an outcome like this proposal is even remotely likely. Requests to Pharmac by Mr Forman for an update on work they are doing on equity in their funding decisions, he says, show they are not doing any work in this regard. They are limiting equity considerations to an outcome focus on whether funded drugs are being accessed across the whole population.