Pharmac Review

Rare Disorders NZ

What is a Rare Disorder?

- There are an estimated 300,000 New Zealanders (definition of 1 in 2000 or less/ the Pharmac definition 1 in 50,000 is often classed as ultra -rare internationally)
- Many different types (6000-10,000)
- Rare disorders can be genetic (80%), rare cancers and rare infections.
- Rare disorders are chronic, progressive, degenerative, disabling and frequently life-threatening

The Terms of Reference (ToR) for the Pharmac Review Committee. Where do Rare Disorders fit?

The Review is timely as it will be informed by government decisions around health system reforms and take these into account in considering the ongoing role of Pharmac.

The government is making crucial and far-reaching changes to our healthcare system without acknowledgment of persons with Rare Disorders as a health priority.

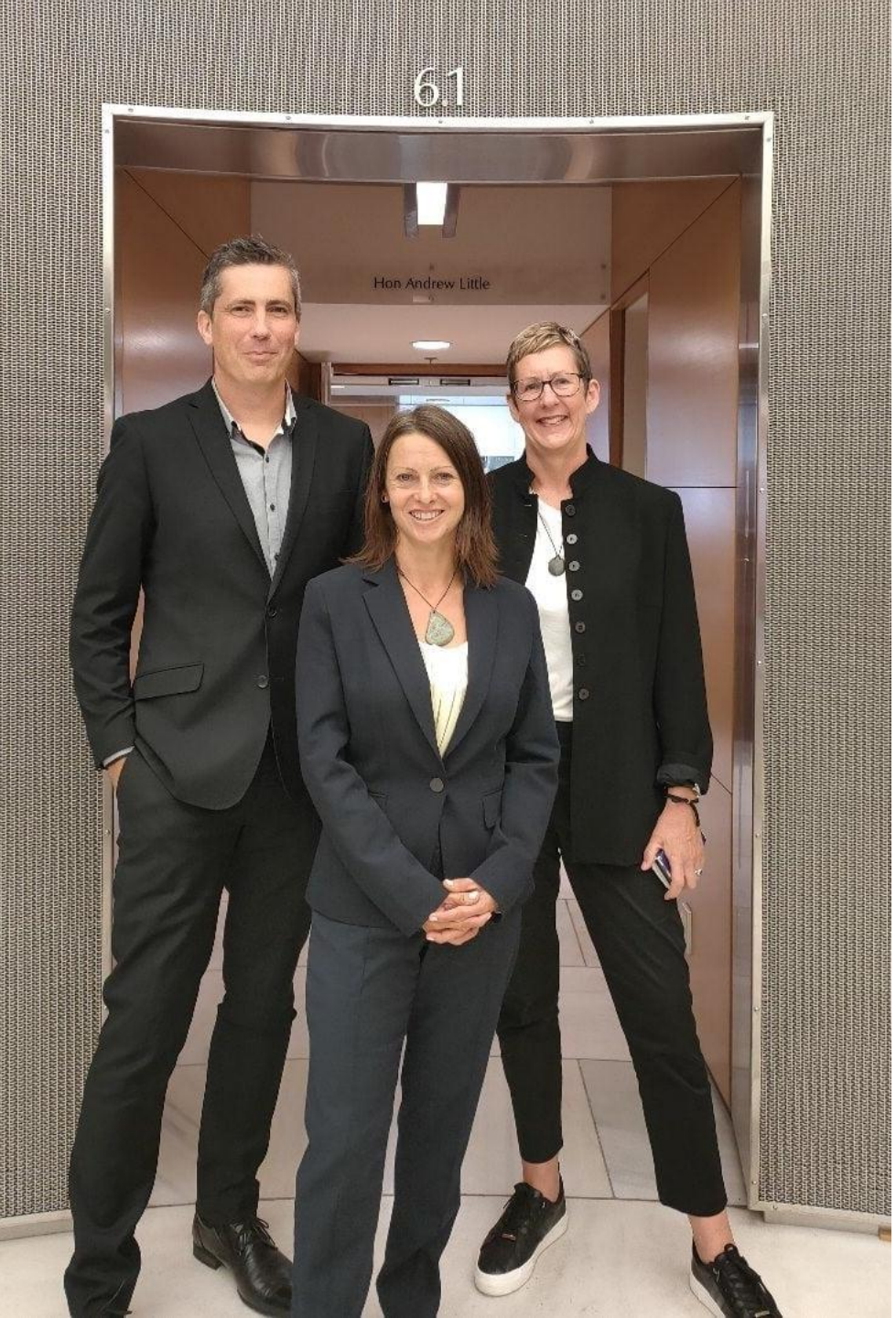
How will PHARMAC tackle rare disorders when it is not specifically acknowledged in health system reforms?

Scope of the Review - will cover two key issues;

1. *How well Pharmac performs against its **current objectives** and whether and how its performance against these could be improved?*
    - EQUITY: When you look at the *2020 Pharmac Statement of Intent* and set objectives rare disorders are mentioned in opening statement once. If there is no specific performance objective set then how can there be improvements? What is not counted doesn't count!
2. *Whether Pharmac's current objectives maximise its potential to improve health outcomes for **all New Zealanders** as part of the wider health system, and whether and how these should be changed.*
    - All New Zealanders, MUST include the 300,000 New Zealanders with a rare disorder. (only 4-5% have a treatment available, it is not an endless stream)
    - *Current model for assessing benefits and costs not fit for purpose. Alternative assessment models are used by Scotland, Australia and UK*

Challenges

- New ‘breakthrough’ genetic and precision medicines cost more yet offer life-changing outcomes with reduced burden of disease. PHARMAC is not future proofed to deal with what is to come.
- Apples and Pears; High volume and high-cost treatments need to be assessed very differently than high cost, low volume treatments.
- Small population size mean assessment based mostly on cost savings and cost effectiveness are not ‘fit for purpose’
- PHARMAC funding needs to be **sufficient** to incorporate realistic needs of the country or the model cannot work for all
- Lack of rare disorder data being collected in NZ is a barrier to transparency, budget reports and therefore resourcing
- Only 2 modern medicines to treat rare disease were publicly funded between 2011-2018 in NZ out of 36 launched in the OECD.



Human Rights

Call for UN General Assembly Resolution on Addressing the Challenges of Persons Living with a Rare Disease and their families

Fight against Stigma, Discrimination, Exclusion, and Marginalisation- a need to improve awareness and status of this vulnerable population.

Marginalised ethnicities living with rare disorders, particularly Māori and Pacific peoples in low socio-economic populations face insurmountable barriers in access to medicines

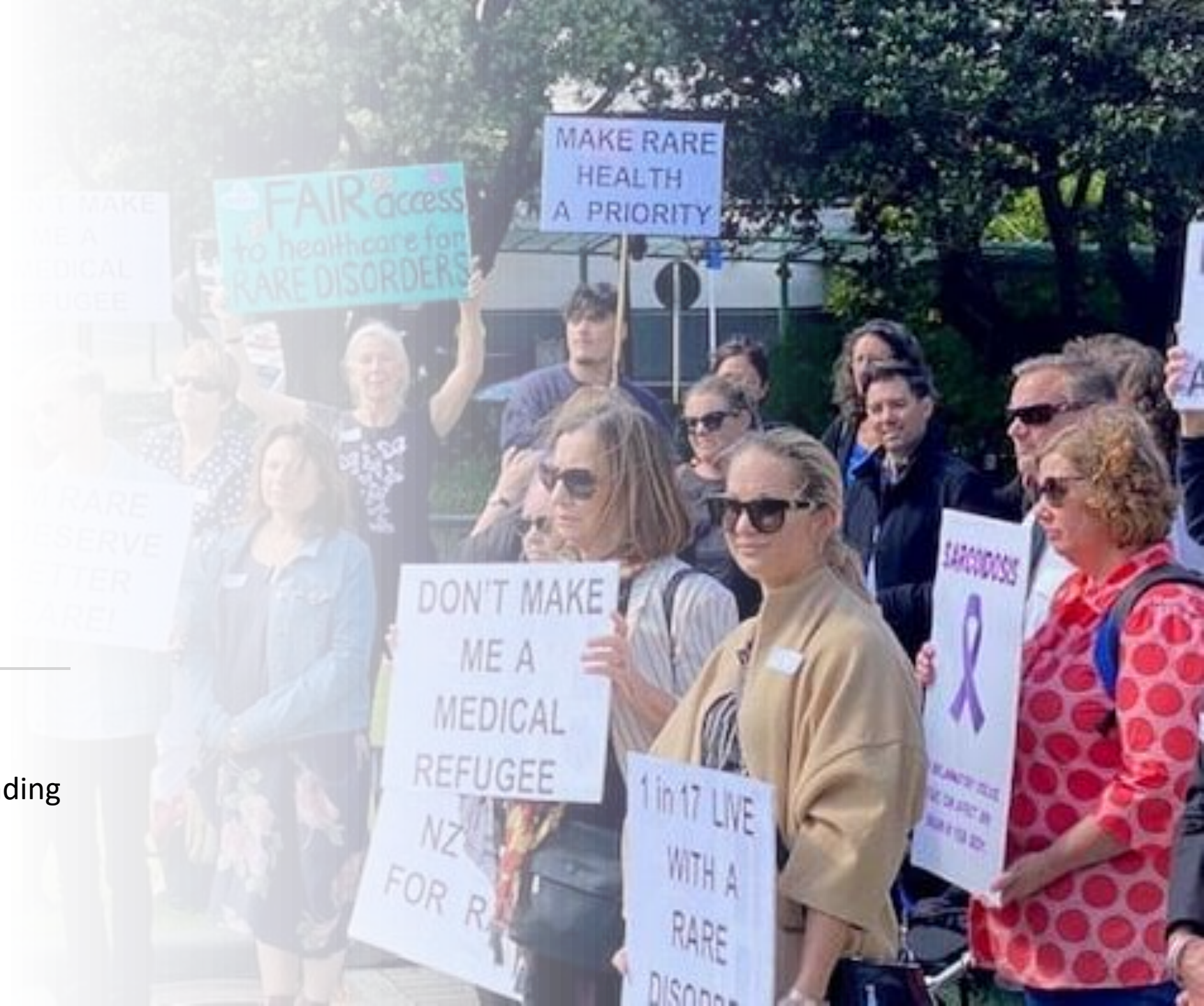
APEC Rare Disease Action Plan; no current objectives in NZ, Why Not?

Universal Declaration of Human Rights + Report by the UN High Commissioner for Human Rights + Human Rights Council on access to medicines and vaccines

Social development challenges of access benefits and services not only medicines

Solutions

- Inclusion of consumer voice
- Models for early access
- Credible Medicine Strategy
- Cost-Risk sharing models
- Co-payments models
- Reduced criteria to match the challenges of small populations
- Rapid Access Scheme
- Collect data on rare disorders
- PHARMAC to have separate funding to DHB's/ Health NZ



Costs vs Savings – Data

- Productivity loss in USA include $135 billion (2 Billion for NZ?)from adults with RD whose disease progression and diagnoses require time away from the workplace and $152 billion (2 Billion) from their caregivers.

2019 NZ Voice of Rare Disorders Survey

- High utilisation of healthcare services including specialist and GP visits, and diagnostic tests. **One in three people were in hospital for an average of 16 days per year**. **One in twenty people were in ICU for an average of eight days per year**. 38% had an emergency hospital visit in the previous six months.
- There were **almost no effective treatments accessible** for the majority of people other than for reducing inflammation.
- **Full employment and education are a challenge** for people living with a rare disorder and their carers: this may require modifying work arrangements through part-time contracting; or continuing absence from school for children. 73% had to stop work due to their health.

Data limitations mean **the full cost** picture, particularly for rare disorders, is not available for assessment considerations

PHARMAC review submission questions and response from Rare Disorder NZ

3. What are the challenges with PHARMAC's functions for funding medicines and devices?

The current PHARMAC model is not fit-for-purpose as it does not fund the growing needs of those with rare disorders due to small population and high-cost medications.

4. What do you think works well with the processes PHARMAC uses to assess the funding of medicines and medical devices?

- The model works well for low-cost medications for high populations.
- It offers a model to help negotiate reduced costs by bundling medicines or devices and that works in favour of cost savings.

5. What do you think are the barriers to accessing medicines and devices?

There are no alternative assessment pathways for small populations with high-cost, modern, life changing medicines - some are no longer modern as they have been on PHARMAC's waiting list for many years. This is a barrier to those with a rare disorder.

6. Is there any other country that does it better? What is it that it does better and would any of those systems apply here.

The following models operate better for people living with a rare disorder:

- England NICE Model;
- Australia Life Saving Drugs Program;
- Scotland PACE.

These alternative models use assessment criteria which recognise the challenges accessing medicines for small populations such as those with rare disorders. These challenges include smaller populations for clinical trials, higher costs to complete clinical trials, less people to pay for the medicines once reached production and therefore higher prices.

7. How might PHARMAC look in the future? And what needs to change for this to happen?

- PHARMAC needs to be inclusive of those with rare disorders (small populations)
- The PHARMAC of the future would look at other financial models for rare disorders such as cost/risk sharing, co-payment schemes, bundling and international partnerships.
- PHARMAC must have an effective process for high-cost medication for rare disorders (small populations)
- Reduce the barriers that exist and offer more flexible criteria to consider the challenges and barriers for pharmaceutical companies, including removing the Medsafe approval process when a medication has been assessed as safe in other OECD countries.
- Increased patient input and voice at pivotal assessment stages, add clinical expertise on specific disease types to assessment model and include the patient narrative. Reduce the current expectation of clinical trial data.
- Transparency – Measurement of those accessing any rare disorder medicine to show cost (rare disorder data).
- New Zealand needs a Medicines Strategy.

8. Are there additional or different things that PHARMAC should be doing?

- Increased patient voice at key stages of the assessment model.
- Mechanisms and systems within the PHARMAC model to collect transparent accumulated rare disorder data, in alignment with the wider health data systems.
- Significant ringfenced budget for the medicine needs of all New Zealanders separated from other government departments.

Rare Disorders NZ Medicine Access Groups

- Duchenne
- Mal De Embarquement Syndrome
- PKU NZ