VALUING LIFE
NEW ZEALAND MEDICINES ACCESS SUMMIT

Doctors' Experience

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DISCLAIMER

- The views and opinions presented are my own
- I have received consultancy fees from Biogen and Roche for Advisory Board and educational speaking engagements.

THE RARE DISORDERS SPACE

Over 6000 clinically defined rare disorders

72% genetic

70% start in childhood

3.5 - 5.9% of the world population

300 million people in the world living with a rare disorders

New Zealand Rare Disorders Strategy under development, to improve the lives of people living with rare disorders

Rare disorder are estimated to make up 6% of New Zealand population

300,000 people living in New Zealand with a rare disorder

SPINAL MUSCULAR ATROPHY

- A severe neurogenetic disorder, 1:10 000
- Progressive muscle weakness.
- Without treatment it is the most common genetic cause of death in infancy
- Two funded life saving therapies funded in 2023
- Newborn screening programme - 2025
- But
  - No access to single dose gene therapy
  - No funding for affected adults
    - Both available in Australia since 2022
  - No new funded therapies in the neurological or rare disorders space in NZ in 2024-2025

DUCHENNE MUSCULAR DYSTROPHY

Progressive muscle wasting disease with death age 20-30 years - 1/3500 males

8 new therapies funded internationally over the last 5 years

- **None** have an application for registration or reimbursement in NZ
- Prednisone is our only therapy

EPILEPSY

1% of the population

1 in 3 have drug resistant epilepsy

In the last 10 years:

- Australia funded 5 new antiseizure medications
- NZ funded 1

2024 LEARNINGS

- The process in New Zealand needs to be faster ✗
- PTAC committees need provision for expert consultation ✓
- Needs improved communication with pharmaceutical companies ?
- Better engagement with the affected community ✓
- Improved transparency around prioritisation of funding ✗
- Reduce the time between a recommendation for funding and reimbursement ✗
- New Zealand falling to the bottom compared with other OECD countries

WHY?

- Increasing move towards high-cost medicines, especially genetic or “gene therapies”
- Extraordinarily high bar for funding to be considered
  - With a focus on cost, medicines need to not just work, but work spectacularly for the price
  - Medicines that slow decline or reduce symptoms don’t meet the threshold for consideration
  - Largely no hope for high-cost medicines for rare disorders
- Not perceived as a viable market by Pharma
- Inadequate horizon scanning
- Lack of clinical trials infrastructure, value and investment