VALUING LIFE
NEW ZEALAND MEDICINES ACCESS SUMMIT

New Zealand Doctors Journey

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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

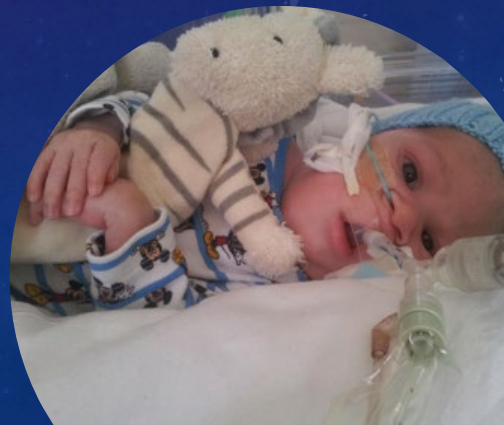
New Zealand Rare Disorders Strategy under development, to improve the lives of people living with rare disorders

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

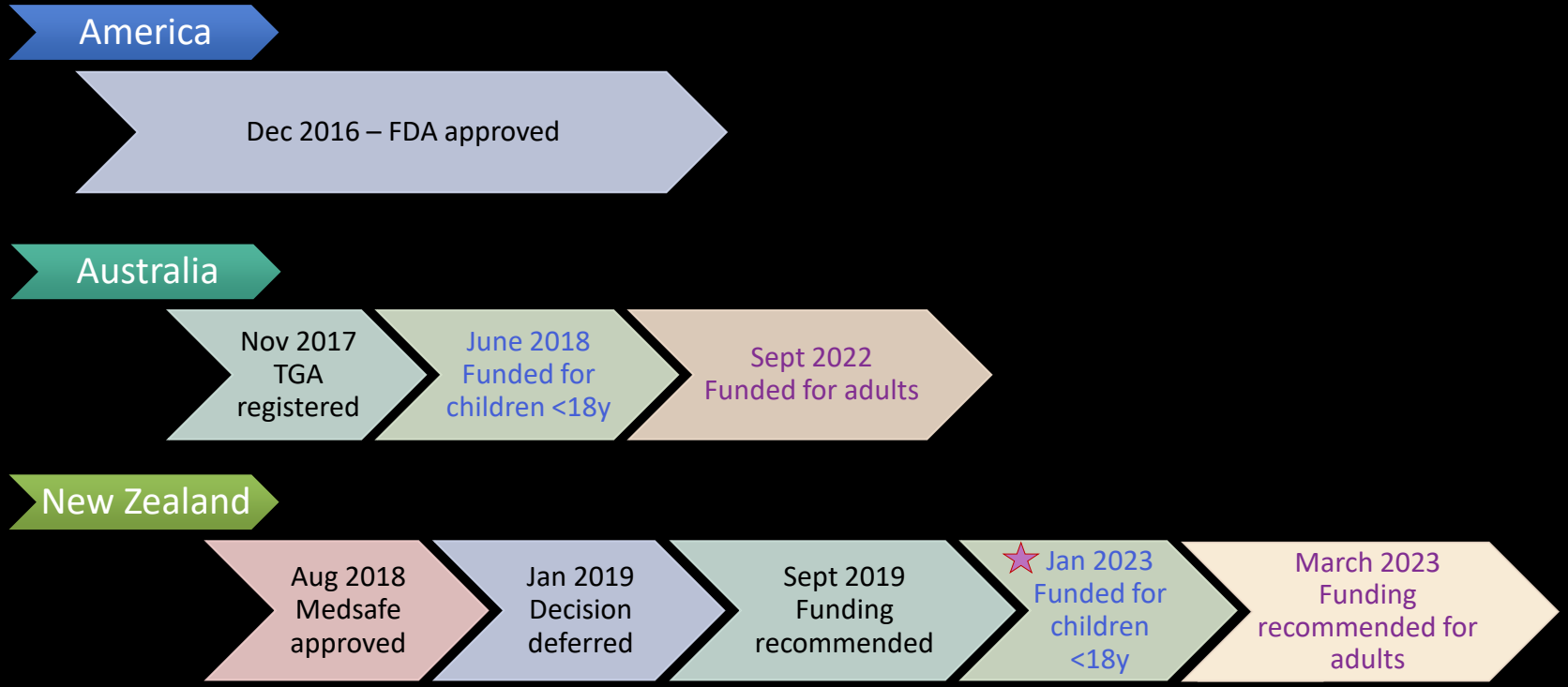
300,000 people living in New Zealand with a rare disorder

SPINAL MUSCULAR ATROPHY

- A neuromuscular disease of infancy, childhood and adulthood
- Affects motor nerves of the spinal cord
- Progressive muscle weakness.
- High morbidity and mortality
- Incidence ~ 1:10,000



Without treatment it is the most common genetic cause of death in infancy



51 countries had formal reimbursement programmes in place

10 families left New Zealand to access treatment elsewhere

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

FUNDING IN THE RARE DISORDERS SPACE

- Pharmaceutical companies already disincentivised to considered reimbursement in New Zealand
  - Low volume market
- Increasing move towards very high cost medicines
  - Gene therapy for SMA funded in Australia since May 2022
  - Medsafe approval granted Feb 2024
- Requires consideration of a different funding model
  - Consider long term, not short term, cost-benefit equations
  - Needs to take broader societal perspective into account.