



Australian
Neuromuscular
Disease Registry

ANMDR
funding
support



Pharma funding support



Philanthropic funding support
Save our Sons & MDNSW



TREAT-NMD reimbursement

ANMDR: relaunched 2019

HREC:54969



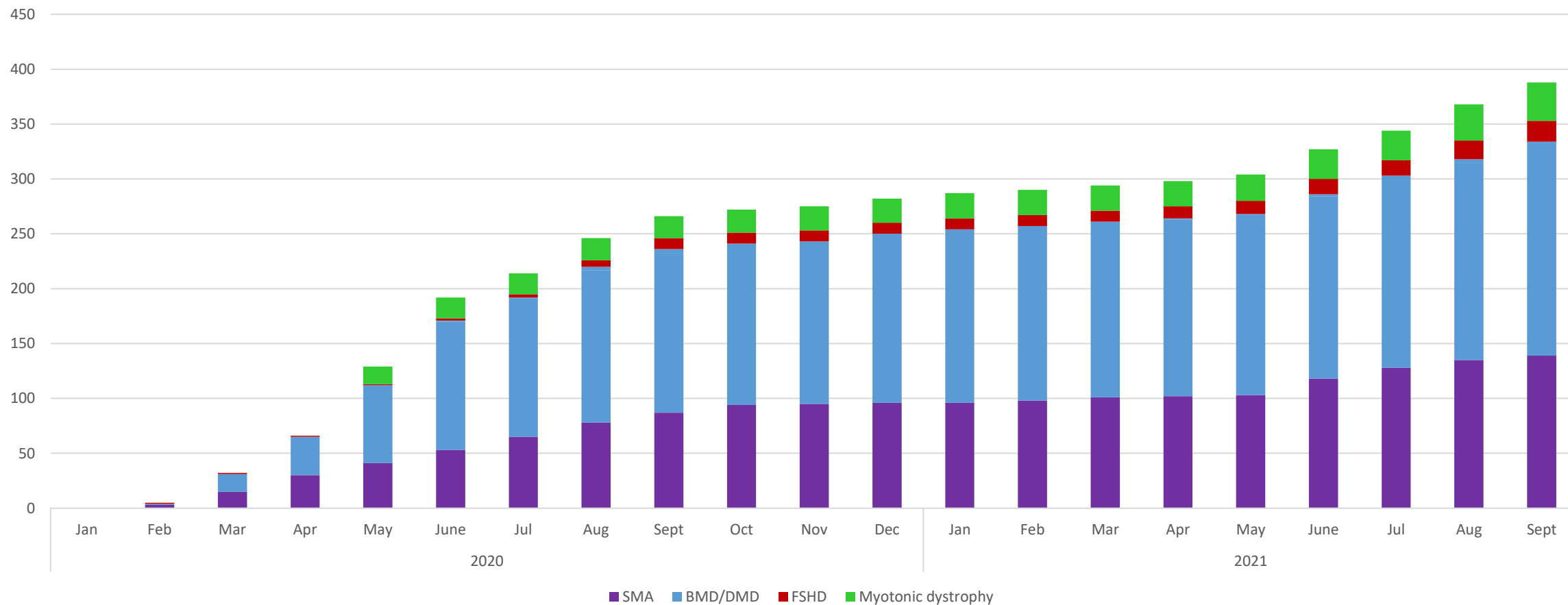
Australian
Neuromuscular
Disease Registry

The ANMDR is part of the TREAT-NMD Alliance (<http://www.treat-nmd.eu/>), a global network of national patient registries for neuromuscular disorders.



Registry Oct 2021

ANMDR recruitment by condition



ANMDR

Community Register

- Held by support group
- Data on members of the NM community
- Often Used for Advocacy

Australian Neuromuscular Disease Registry

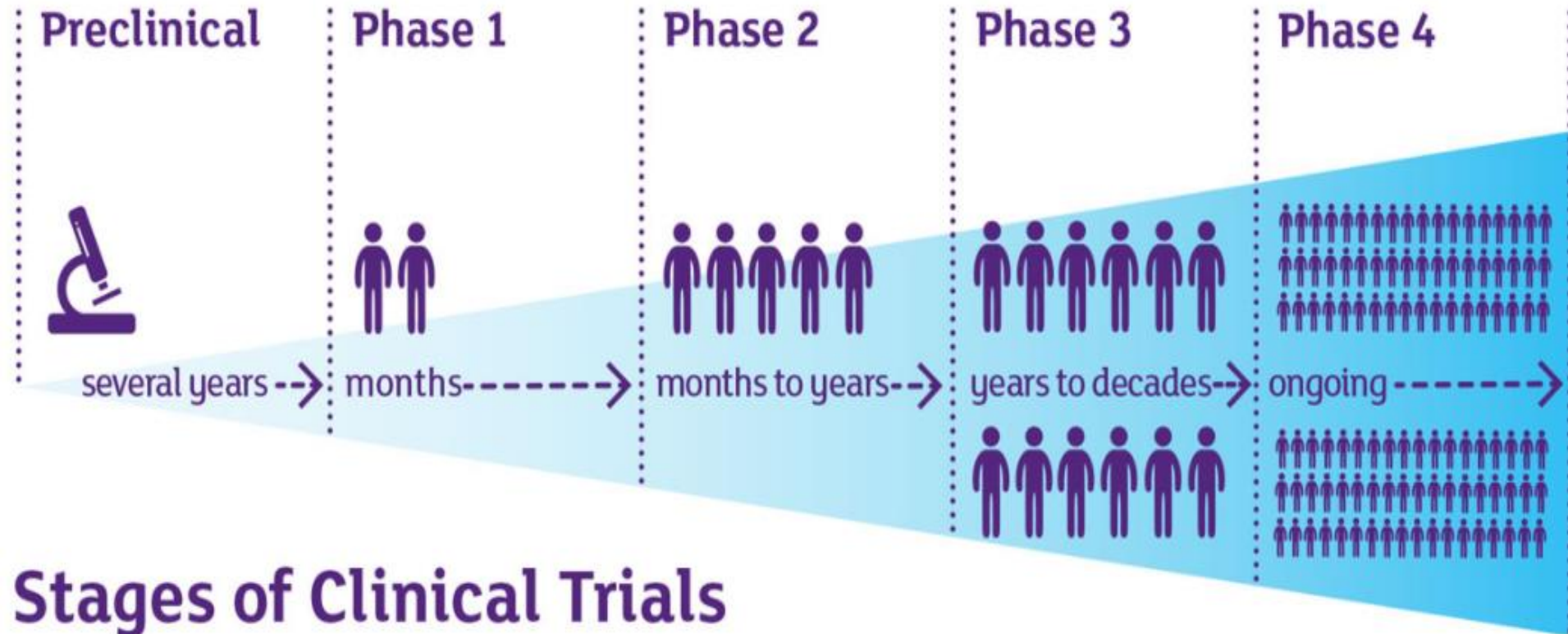
- SMA
- DMD
- Myotonic dystrophy
- FSHD
- LGMD
- Myotubular myopathy
- Congenital Muscular dystrophy
- CMT

Why a Registry?

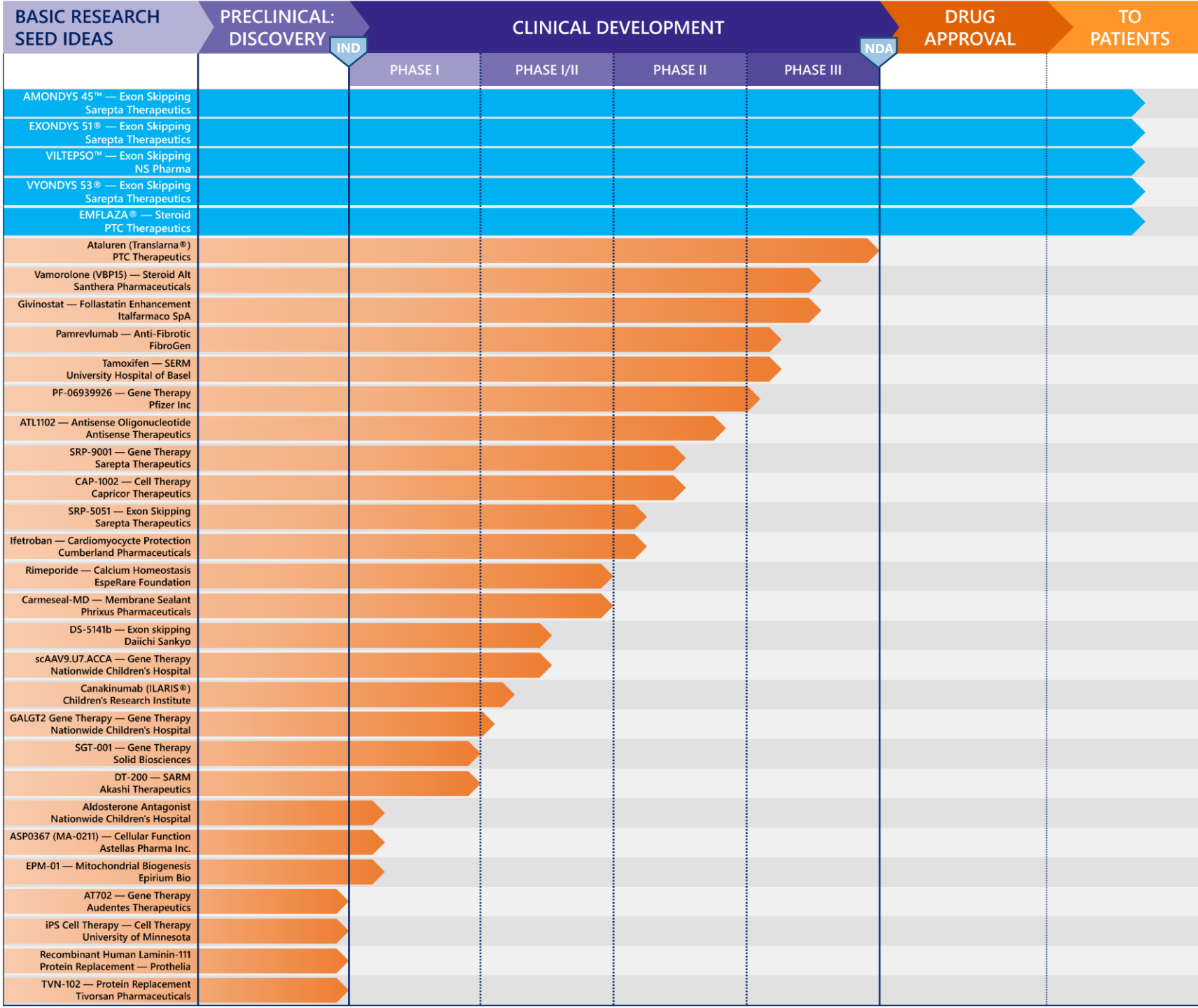
- **Clinical trial readiness (bring to Australia)**
- **Learn about natural history of condition (how it progresses over time)**
- **How to best monitor NMD**
- **How to best treat NMD**
- **Eventually to be “Phase 4” – results in actual practice**



How do clinical trials progress?



Stages of Clinical Trials



IND: Investigational
new drug *

NDA – New drug
application*

How do we get clinical trial ready?

- Be on the Registry
- Genetic confirmation of your diagnosis
- Data updated annually
 - twice a year for patients with SMA
 - standardized and validated measures
- Robust longitudinal data collection
- We need to know where you are in Australia



Registry fatigue

Uses of the data?

Enquiries (International) - 11

New Data set build – SMA

DMD

LGMD (pilot testing site/working gp)

“Potential to use the ANMDR as a case study/reference site for future implementations.”

PBAC – SMA stake holder meeting

Registry data for post- marketing surveillance/licensing

Ultra Rare subgroup – develop data set/add to ANMDR



Why consider registering?

- **Very rapidly changing needs for NM community – we can respond with Real World Data/Evidence that is local**
- **Data (quality) essential for regulatory requirements**
- **Capturing natural history and new natural history**
- **Measure effectiveness of interventions**
- **Inform standards of care**
- **Power of NM community**



Why a Registry?

New disease modifying therapies
require high quality data

Good quality data informs
initiation and continuation of
therapy

ANMDR – can inform new NM trajectory

- High quality, reliable data essential – new disease modifying therapies
- Help identify potential outcome variation of treatment/s
- Respond with rapid updates as Real World Evidence becomes available
- Inform scientific, clinical and health policy communities
- Powerful tool to observe course of NM conditions – monitoring outside clinical trial setting (Phase 4)
- Research tool
- **Support patient advocacy**



Australian Neuromuscular Disease Registry

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The Australian Neuromuscular Disease Registry (ANMDR) is an Australia-wide registry of people diagnosed with a neuromuscular disease. It collects important medical information from adult and child patients across the country to improve the understanding of neuromuscular disease and accelerate the development of new therapies.

[register your interest](#)

Please contact us:



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