

Australian Neuromuscular Disease Registry

# ANMDR funding support

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#### Pharma funding support



Philanthropic funding support

Save our Sons & MDNSW



**TREAT-NMD** reimbursement

## ANMDR: relaunched 2019 HREC:54969

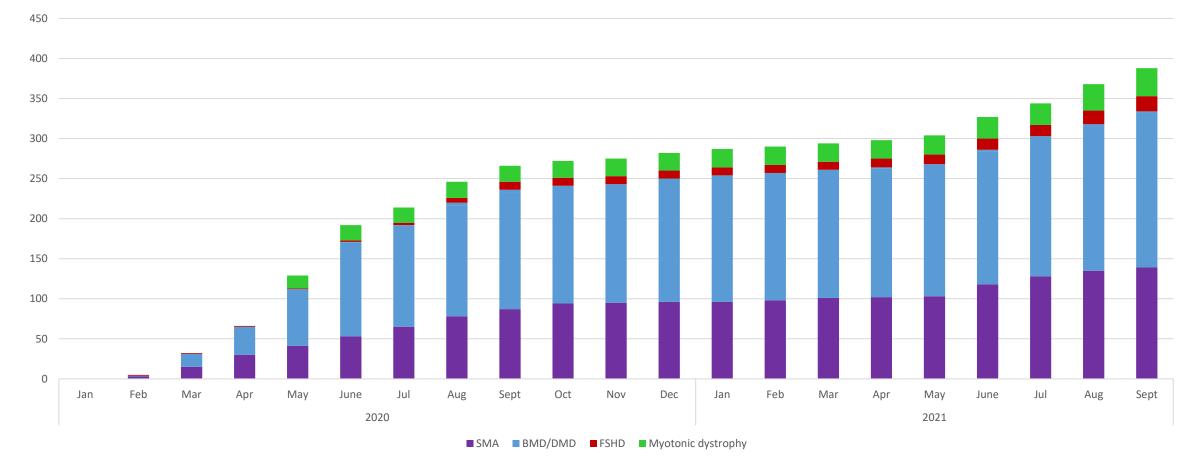


The ANMDR is part of the TREAT-NMD Alliance (<u>http://www.treat-nmd.eu/</u>), a global network of national patient registries for neuromuscular disorders.



## **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

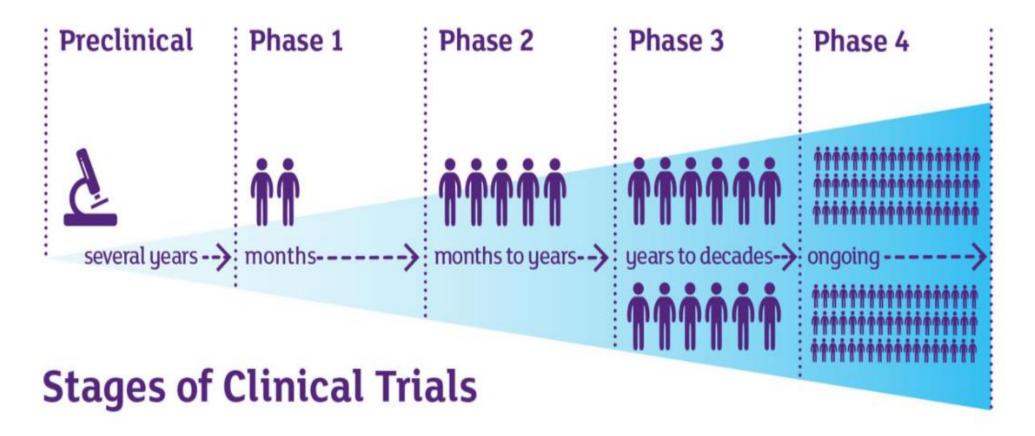
- SMA
- DMD
- Myotonic dystrophy
- FSHD
- LGMD
- Myotubular myopathy
- Congenital Muscular dystrophy
- CMT

# 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?



	BASIC RESEARCH SEED IDEAS	PRECLINICAL: DISCOVERY	CLINICAL DEVELOPMENT				DRUG APPROVAL	TO PATIENTS
			PHASE I	PHASE I/II	PHASE II	PHASE III		
	AMONDYS 45 <sup>™</sup> — Exon Skipping Sarepta Therapeutics							
	EXONDYS 51® — Exon Skipping Sarepta Therapeutics							
	VILTEPSO <sup>™</sup> — Exon Skipping NS Pharma							
IND: Investigational	VYONDYS 53® — Exon Skipping Sarepta Therapeutics							
new drug *	EMFLAZA® — Steroid PTC Therapeutics							
5	Ataluren (Translarna®) PTC Therapeutics							
	Vamorolone (VBP15) — Steroid Alt Santhera Pharmaceuticals							
	Givinostat — Follastatin Enhancement Italfarmaco SpA							
	Pamrevlumab — Anti-Fibrotic FibroGen							
	Tamoxifen — SERM University Hospital of Basel							
	PF-06939926 — Gene Therapy Pfizer Inc							
	ATL1102 — Antisense Oligonucleotide Antisense Therapeutics							
	SRP-9001 — Gene Therapy Sarepta Therapeutics							
	CAP-1002 — Cell Therapy Capricor Therapeutics							
	SRP-5051 — Exon Skipping Sarepta Therapeutics							
	Ifetroban — Cardiomyocycte Protection Cumberland Pharmaceuticals							
	Rimeporide — Calcium Homeostasis EspeRare Foundation							
	Carmeseal-MD — Membrane Sealant Phrixus Pharmaceuticals							
NDA – New drug	DS-5141b — Exon skipping Daiichi Sankyo							
application*	scAAV9.U7.ACCA — Gene Therapy Nationwide Children's Hospital							
	Canakinumab (ILARIS®) Children's Research Institute							
	GALGT2 Gene Therapy — Gene Therapy Nationwide Children's Hospital							
	SGT-001 — Gene Therapy Solid Biosciences							
	DT-200 — SARM Akashi Therapeutics							
	Aldosterone Antagonist Nationwide Children's Hospital							
	ASP0367 (MA-0211) — Cellular Function Astellas Pharma Inc.							
	EPM-01 — Mitochondrial Biogenesis Epirium Bio							
	AT702 — Gene Therapy Audentes Therapeutics							
	iPS Cell Therapy — Cell Therapy University of Minnesota							
	Recombinant Human Laminin-111 Protein Replacement — Prothelia							
	TVN-102 — Protein Replacement Tivorsan Pharmaceuticals							

# 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

tion & Research Day 9th

# 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





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





Home

About us Type of diseases Type of diseases

Clinical trial finder

Contact us 🔻

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:

