

Innovative Therapeutic Development Approaches in Neuromuscular Diseases

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Our Mission Is Clear

We are committed to transforming the lives of people living with neuromuscular disease.



We're Innovative In Our Approach to Transforming Lives



Innovations in Science

We're helping accelerate the development of treatments and cures.



Innovations in Care

We provide programs and services that transform the lives of people living with neuromuscular disease.



Innovations in Science

RESEARCH

We're the largest source of funding for neuromuscular disease research outside the federal government for NMDs--**\$1B+** since we started 68 years ago

TREATMENTS

Our research is directly linked to approved, life-changing therapies across multiple neuromuscular diseases

TECHNOLOGY

Our Neuromuscular Observational Research (MOVR) data hub gathers longitudinal clinical and genomic data to help accelerate therapy development and improve health outcomes



Innovations in Care

CARE CENTERS

We support the largest network of centers providing expert care at top-tier institutions

RESOURCES

We provide one-to-one specialist support and host educational and social events and programs for individuals, families, researchers and health care professionals

RECREATION

Vital life skills and independence provided through summer camps and other recreational facilities – at no cost to families

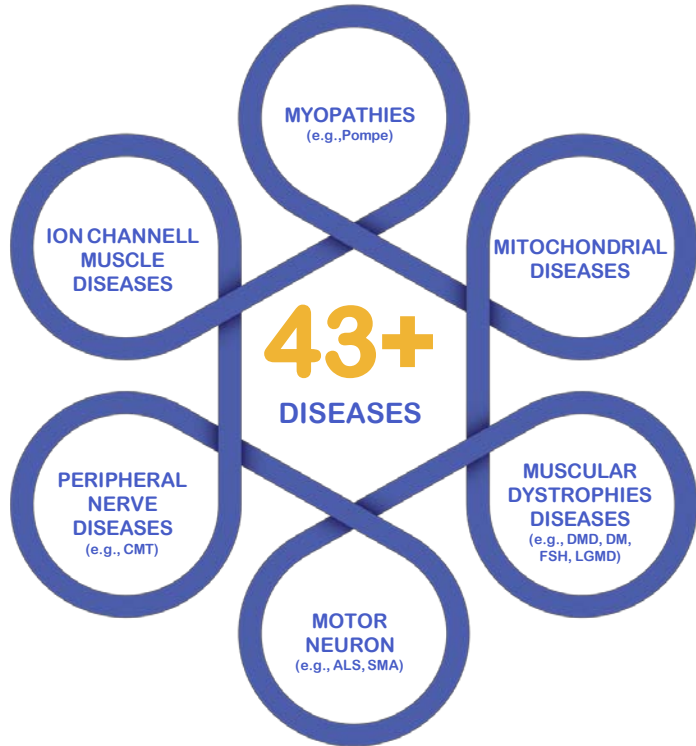
An Unprecedented Number of FDA Approved Treatments in Recent Years:



*Supported by MDA funding.

Eight (8) new therapies in the past four (4) years

Unique Challenges in Neuromuscular Diseases



- Lack of disease understanding
- Lack of biomarkers
- Extensive heterogeneity in disease progression and manifestation
- Variety of genetic and mitochondrial underpinnings
- Often extremely small patient population
- High severity of disease
- Pediatric populations
- Archaic endpoints

Innovative Approaches to Therapeutic Development



- Platform trial designs
- Adaptive clinical trial designs
- Historical controls (or at least non-placebo controls, if possible)
- Open-label studies
- Broad trial eligibility criteria
- Expanded access
- Remote/mobile data collection
- Patient-preferred endpoints
- Rapid trial timelines



Requests of FDA:



250+

unique assets in
preclinical and
clinical
development

150+

unique assets in
preclinical
development

60+

companies
active in Phase I
to Phase III
development

22

products in Phase III
– 7 in ALS and 6 in
DMD

- Structured, transparent, and more extensive disease-specific guidance development
- Full implementation of flexible mechanisms endorsed in guidances
- Review division consistency
- Patient and advocate engagement within new CDER structure
- Continued collaboration with the patient community

How Will MDA Help?

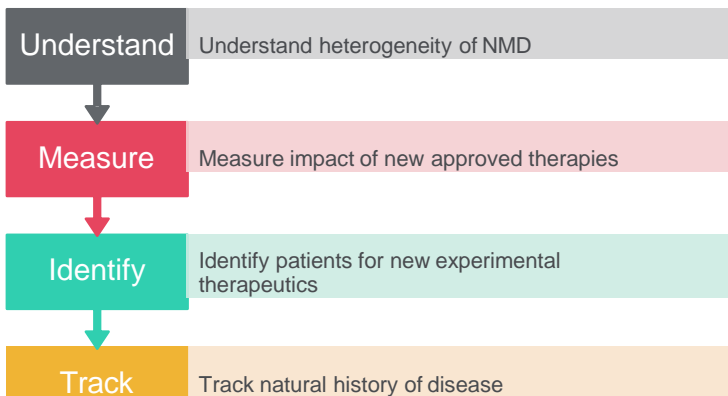


- Patient education
- Support of and participation in patient participation opportunities
- Policy and regulatory support
- Externally-led Patient-Focused Drug Development meetings
- Patient preference data collection through our OneVoice program
- Longitudinal natural history data collection through MOVR



MOVR Data Hub

Gathers genomic and longitudinal clinical data across diseases to help accelerate therapy development and improve health outcomes





Thank You

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