Innovative Therapeutic Development Approaches in Neuromuscular Diseases

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Muscular Dystrophy Association
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
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:

2015*
KEVEYIS®
(dichorphenaimde)
Periodic Paralysis

2016*
EXONDYS 51®
(eteplirsen)
Duchenne Muscular Dystrophy (DMD)

2016*
SPINRAZA®
nusinersen
Spinal Muscular Atrophy (SMA)

2017*
EMFAZA®
(deflazacort)
Duchenne Muscular Dystrophy (DMD)

2017
RADIÇAVA®
edaravone
ALS

2017
SOLIRIS®
(eculizumab)
Myasthenia Gravis

2017
FIRDAPSE®
amifampridine
LEMS
(Lambert-Eaton myasthenic syndrome)

2018
Zolgensma
(onasemnogene abeparvovec-xioi)
Spinal Muscular Atrophy (SMA)

2019
Zolgensma
(onasemnogene abeparvovec-xioi)
Spinal Muscular Atrophy (SMA)

*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:

• 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**

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<thead>
<tr>
<th>Understand</th>
<th>Understand heterogeneity of NMD</th>
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<tr>
<td>Measure</td>
<td>Measure impact of new approved therapies</td>
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<tr>
<td>Identify</td>
<td>Identify patients for new experimental therapeutics</td>
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<tr>
<td>Track</td>
<td>Track natural history of disease</td>
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Thank You

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