DUCHENNE MUSCULAR DYSTROPHY RESEARCH PROGRAM

CDMRP
DEPARTMENT OF DEFENSE
CONGRESSIONALLY DIRECTED MEDICAL RESEARCH PROGRAMS

MISSION: To support discovery, development and delivery of therapeutics for Duchenne muscular dystrophy at all stages of the disease for the benefit of military Families and the general public

Congressional Appropriations FY11-FY24:

$79.6M total

"I feel fortunate to be part of DMDRP and have my voice heard by the researchers and doctors working toward better care and treatments for people living with Duchenne muscular dystrophy. I'm grateful that the research supported by the DMDRP encompasses all aspects of care and efforts toward a better understanding of the disease, and I'm especially excited about research toward a cure, as it's something I've prayed for and dreamt of for 18 years."

Josh Argall,
CureDuchenne,
FY21-FY22 Peer Reviewer

SCOPE OF THE PROBLEM

About **20,000 children** diagnosed each year[1]

Average **life expectancy is 28 years**[2]

There is no cure: DMD is always fatal

Affects ~1 of every **5,000 male infants**[1]

RELEVANCE TO MILITARY HEALTH –

Hereditary Progressive & Duchenne/Becker Muscular Dystrophies

DOD Beneficiaries Military Health System Medical Encounters Over a 9-Year Period[3]	Outpatient Encounters	Hospital Bed Days
	115,656	49,371

PROGRAM PRIORITIES

- Accelerate discovery and development of therapeutics with a path to clinical applications
- Advance understanding of the effect of DMD on skeletal muscle, heart, bone, central nervous system, and gastrointestinal system across the life span of Duchenne and Becker muscular dystrophy
- Enhance DMD research capacity

[1] Venugopal V and Pavlakis S. Duchenne Muscular Dystrophy StatPearls (Internet). 2023.
[2] Broomfield J, Hill M, et al. Neurology, 97, no. 23, 2021: e2304-2314.
[3] MHS data from the Defense Medical Surveillance System. The Armed Forces Health Surveillance Branch, Defense Health Agency, Silver Spring, Maryland, 2013-2022, January 2024.

For more information, visit: https://cdmrp.health.mil/dmdrp

PROGRAM IMPACT AND OUTCOMES

Ongoing Research Spans Early Ideas Through Clinical Trials

Early Stage Ideas

- Gene therapy to reduce muscle fibrosis and promote muscle regeneration
- Mechanisms underlying severe adverse reactions to adeno-associated virus gene therapy redosing

Translational

- Noninvasive MRI biomarkers of bone quality
- MyoTRIM – a novel protein therapeutic intervention to enhance the repair capacity of skeletal and cardiac muscle

Funded Awards with Clinical Trials

- Modified glucocorticoid regimen to enable exercise training to delay disease progression, reverse secondary effects of disuse and induce beneficial adaptations
- Personalized cardiac risk-assessment models through collection of ECG and MRI data

SUPPORTING THE DMD COMMUNITY THROUGH COLLABORATION

The DMDRP is actively engaged with the federal advisory Muscular Dystrophy Coordinating Committee

DMDRP | Administration for Community Living | CDC | FDA | Health Resources and Services Administration | Medicare and Medicaid Services | Social Security Administration | NIH | Patient Organizations

The MDCC leverages gaps identified in the **2015 MDCC Action Plan** for the Muscular Dystrophies[4]

Understanding Causes | Screening and Diagnosis | Developing Treatments | Preparing for Clinical Trials | Providing Care, Management and Access to Services

[4] Rieff H, et al. *Muscle Nerve*, 53, no. 6, 2016: 839-841.

RESEARCH BREAKTHROUGHS - MAKING A DIFFERENCE

FDA-Approved Drugs

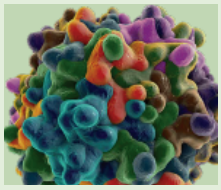
- **Exondys 51®** and **Viltepso** antisense oligonucleotide treatments for skipping over/excluding gene mutations to produce functional dystrophin

- **Agamree 51®**, a non-hormonal steroid drug decreases muscle inflammation with reduced side effects compared to other corticosteroid-based treatments

Transitioned Tools to Industry

- Validation of motor function assessment using **Walk4Me** device to capture 3-D features of gait patterns
- Validation of prognostic assay to measure levels of dystrophin in muscle biopsies

Treatment Approaches Now in Clinical Trials



- **Micro-Dystrophin Gene Transfer** – expression of micro-dystrophin produces functional dystrophin, leading to improved cardiorespiratory and skeletal muscle function

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08/2024