CDMRP Department of Defense

Congressionally Directed Medical Research Programs

DUCHENNE MUSCULAR DYSTROHPY RESEARCH PROGRAM

IMPROVING FUNCTION, QUALITY OF LIFE, AND LIFE SPAN

MISSION: To support discovery and development of therapeutics for Duchenne for the benefit of military beneficiaries and the general public, from the characterization of pathophysiology through rigorous preclinical and clinical studies

FY22 Appropriation
$10M

FY22 Funding Mechanisms: Pre-applications are required; full application is by invitation only

Idea Development Award
$350K

Supports high-impact, new ideas in early development

- Established Investigators
- New Investigators – Early Stage: *within 10 years of first faculty appointment*
- New Investigators – Transitioning: *Bringing new expertise to the field via established investigators in an area other than muscular dystrophy*

New for FY22

Translational Research Award
$1.25M

Supports translational research to move Duchenne Muscular Dystrophy (DMD) research into clinical applications

Early-Career Partnering PI Option
$1.35M

Early-Career Partnering PI Option

An Early-Career Partnering Principal Investigator (PI) Option is being offered to promote enhanced research capacity within the DMD field. If exercised, this option allows 2 awards to be made for funded projects with the Early-Career Investigator named as PI of their own award.

Deadlines

August 3, 2022	November 15, 2022	January 2023	March 2023
Pre-Applications Due	Full Applications Due	Peer Review	Programmatic Review

FY22 Focus Areas:

IDEA DEVELOPMENT AWARD:

Applications must address opportunities and challenges in the development of safe and effective macromolecular and cellular therapies that focus on primary pathology of DMD.

TRANSLATIONAL RESEARCH AWARD:

Applications must address at least one of the FY22 TRA Focus Areas.

- Translational and clinical studies, novel interventions, and drug and biologic delivery technologies designed to improve care and quality of life
- Assessment of clinical trial tools and outcome measures
- Extension or expansion of existing preclinical translational data in support of a specific therapeutic development path

Selected FY18-FY20 Awards:

Early Stage Ideas:

- **Muscle-Targeted Cell-Penetrating Peptides for Delivery of Cas9-RNPs and Modified mRNA to Dystrophic Muscle,** *Dr. Samir El Andaloussi*
- **Targeted Cell-Based Gene Therapies for Persistent Exon Skipping and Dystrophin Restoration in DMD,** *Dr. James Novak*
- **Developing a Duchenne Muscular Dystrophy Therapeutic Agent with a New Base Editing Technology,** *Dr. Shengkan Jin*
- **Can Dystrophin-Replacement Therapies Improve Cognitive Function in DMD? Development of Strategies to Maximize Effectiveness and Avoid Detrimental Effects,** *Dr. Holly Colognato*

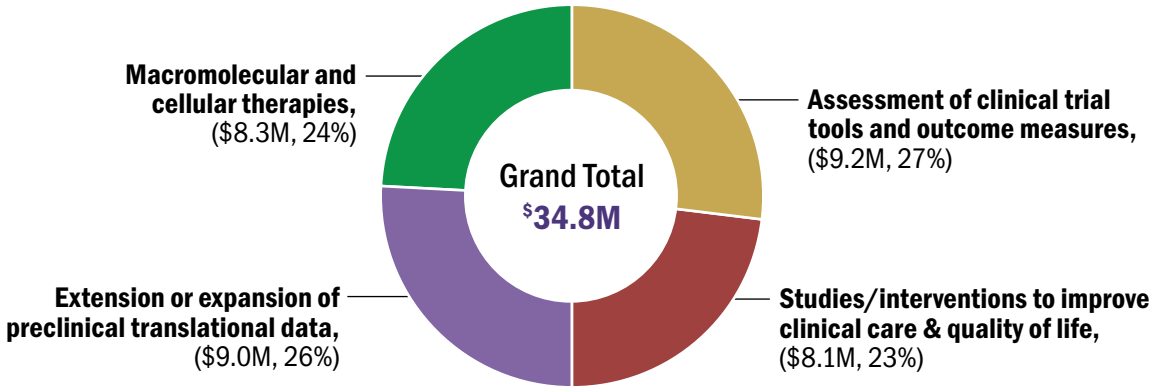
Translational:

- **MRI Biomarkers of Bone Quality in DMD,** *Dr. Rebecca Willcocks and Dr. Chamith Rajapakse*
- **A Novel Office-Based Injectable to Treat Duchenne-Related Fibrosis,** *Dr. Benjamin Cooper and Dr. Ara Nazarian*

Funded Awards with Clinical Trials:

- **Impact and Interplay of Corticosteroid Regimen and Exercise Training on DMD Muscle Function,** *Dr. Tanja Taivassalo Interdisciplinary Collaborator: Dr. Warren Dixon*
- **Assessing Arrhythmic Risk in Adult Patients with Duchenne Muscular Dystrophy,** *Dr. Andreas Barth Interdisciplinary Collaborator: Dr. Natalia Trayanova*

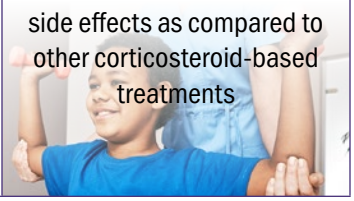
FY11–FY20 Investment by Focus Area



DMDRP Research Breakthroughs

Treatment Approaches Now in Clinical Trials

Micro-Dystrophin Gene Transfer	Vamorolone
Expression of micro-dystrophin produces functional dystrophin, leading to improved cardiorespiratory and skeletal muscle function	Non-hormonal steroid drug that decreases muscle inflammation with reduced side effects as compared to other corticosteroid-based treatments



FDA-Approved Drugs

- **Exondys 51®** (eteplirsen) antisense oligonucleotide treatment for skipping over dystrophin gene mutations in exon 51
- **Viltepso®** (vitolarsen) antisense oligonucleotide treatment for skipping over dystrophin gene mutations in exon 53

Tools That Have Transitioned to Industry

- Validation of prognostic assay to measure levels of dystrophin in muscle biopsies

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

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