DUCHENNE MUSCULAR DYSTROPHY RESEARCH PROGRAM

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VISION *To preserve and improve the function and quality of life, and to extend the life span of all individuals with Duchenne*

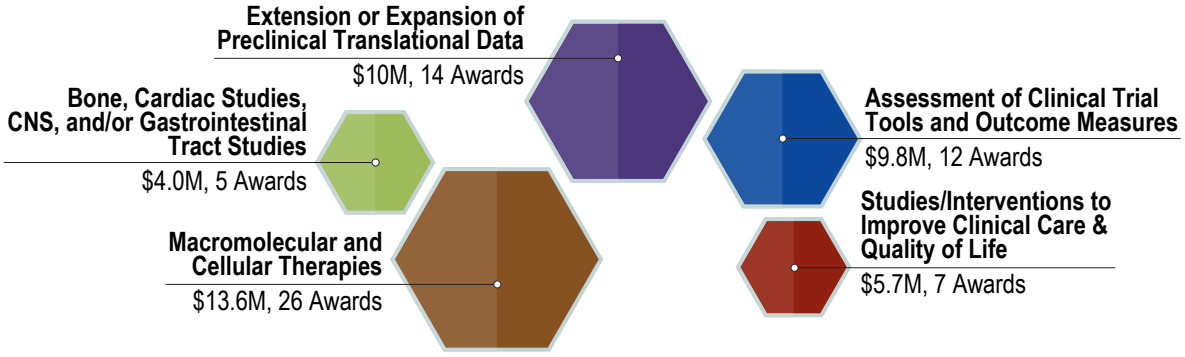
MISSION *To support discovery and development of therapeutics for Duchenne for the benefit of military beneficiaries and the general public, from the characterization of pathophysiology through rigorous preclinical and clinical studies*

PROGRAM HISTORY

Since the inception over the past 11 years since the inception of the Duchenne Muscular Dystrophy Research Program (DMDRP) in 2011, this program has challenged and supported the Duchenne muscular dystrophy (DMD) research community to tackle the difficult questions stemming from therapy development for patients. DMD is the most common childhood form of muscular dystrophy, affecting approximately 1 out of every 5,000 male infants. There is no cure for DMD, and the devastating muscle weakness affecting the skeletal, heart, and respiratory muscles eventually leads to an individual's death before or during their 30s. The DMDRP has received $59.6M in congressional support through FY22, and its current research portfolio of 62 projects includes studies on understanding DMD's effects on bone, heart, and the central nervous system (CNS), research to improve clinical care and quality of life, assessment of clinical trial tools and outcome measures, and preclinical translational research to support therapeutic development.

INVESTMENT STRATEGY

With the lack of any curative treatments, the DMDRP has placed its greatest emphasis on developing or improving treatments and clinical trial readiness. Currently, this program focuses its investments in early ideas that address the challenges and opportunities in the development of safe and effective macromolecular and cellular therapies and in advanced translational projects that have moved beyond the realm of basic research and have the potential to result in near-term impact in clinical research or the clinic. The results of DMDRP-funded research are analyzed for their impact in addressing the program's goals, mission, and vision, and the DMDRP revises its investment strategy as needed.



DMDRP Portfolio Investment FY11-FY21

2021 Congressional Appropriations, Research Investment, and Withholds and Management Costs

Congressional Appropriations	*Research Investment*		*Withholds and Management Costs*	
$10M	Idea Development Award – New & Established Investigators	$5,334,705	USAMRDC	$193,340
	Translational Research Award	$3,469,468	SBIR/STTR	$333,000
	Modification to ongoing awards	$8,106	Mgt Costs (6.98%)	$661,382
Total: $10M		**Total:** $8,812,279		**Total:** $1,187,722

EARLY STAGE IDEAS

Muscle-Targeted Cell-Penetrating Peptides for Delivery of Cas9-RNPs and Modified mRNA to Dystrophic Muscle

Dr. Samir El Andaloussi

Explores a new generation vector system based on cell-penetrating peptides to achieve targeted delivery of disease-relevant biotherapeutics Cas9-RNPs and microdystrophin mRNA in DMD in vitro models and in DMD mice.

Novel Therapeutic Strategy to Achieve Upregulation of Dystrophin Isoforms

Dr. Monkol Lek

Design of a CRISPR gene-editing construct to boost dystrophin levels in patients who express residual or low levels of dystrophin. Especially useful for older patients and those with mild DMD.

Targeted Cell-Based Gene Therapies for Persistent Exon Skipping and Dystrophin Restoration in DMD

Dr. James Novak

Explores the potential clinical application of a cell-based gene therapy delivery strategy using macrophages to continuously deliver antisense oligonucleotides to damaged muscle tissue and improve exon skipping therapies.

Irisin and Therapeutics for Duchenne Muscular Dystrophy

Dr. Bruce Spiegelman

Irisin is a natural hormone secreted from muscle that is increased with endurance exercise. Conducting preclinical studies evaluating whether irisin can improve skeletal muscle and cardiac function in DMD mice.

TRANSLATIONAL

MRI Biomarkers of Bone Quality in DMD

Dr. Rebecca Willcocks and Dr. Chamith Rajapakse

Development of magnetic resonance imaging (MRI) measures of bone quality in individuals with DMD as biomarkers for future fracture treatment and fracture prevention clinical trials in DMD. Would provide more accurate assessment of fracture risk as well as evaluation tools for bone-targeted therapeutics.

A Novel Office-Based Injectable to Treat Duchenne-Related Fibrosis

Dr. Benjamin Cooper and Dr. Ara Nazarian

Translation of a novel relaxin-based injectable treatment to mitigate joint contractures due to fibrosis. Project includes all work needed to prepare for human clinical trials for this product.

CLINICAL TRIALS

Impact and Interplay of Corticosteroid Regimen and Exercise Training on DMD Muscle Function

Dr. Tanja Taivassalo and Dr. Warren Dixon

Clinical trial testing two new therapeutic strategies for patients with DMD: a new low-dose weekend regimen of corticosteroids; and a comprehensive exercise training program. The exercise training program includes a new device, Therex-DMD, which allows for therapeutic exercise within the lower extremities for boys living with DMD.

Assessing Arrhythmic Risk in Adult Patients with Duchenne Muscular Dystrophy

Dr. Andreas Barth and Dr. Natalia Trayanova

Clinical trial to study the natural history of rhythm abnormalities in DMD by implanting miniaturized cardiac monitors in DMD patients at risk for developing potentially life-threatening heart rhythm disorders. A better understanding of arrhythmias could improve patient care by preventing sudden cardiac death.

Jeff Bigelow, Parent Project Muscular Dystrophy, Consumer Peer Reviewer FY20

"Being a member of the review process was exciting. It was invigorating to read research proposals aimed at improving life for my boy and other boys with DMD. It was encouraging to see people dedicating their lives and careers towards making life better for my boy. I know research treatments will be developed to change the life course and day-to-day suffering experienced by my son Henri and other boys with DMD and their families. Great minds are out there, horizons are opening, and this hopeless disease is becoming more hopeful."