# CDMRP: From Advocacy to Impact

## How this community turned stories into science

Parent Project Muscular Dystrophy

JOIN THE FIGHT. END DUCHENNE.

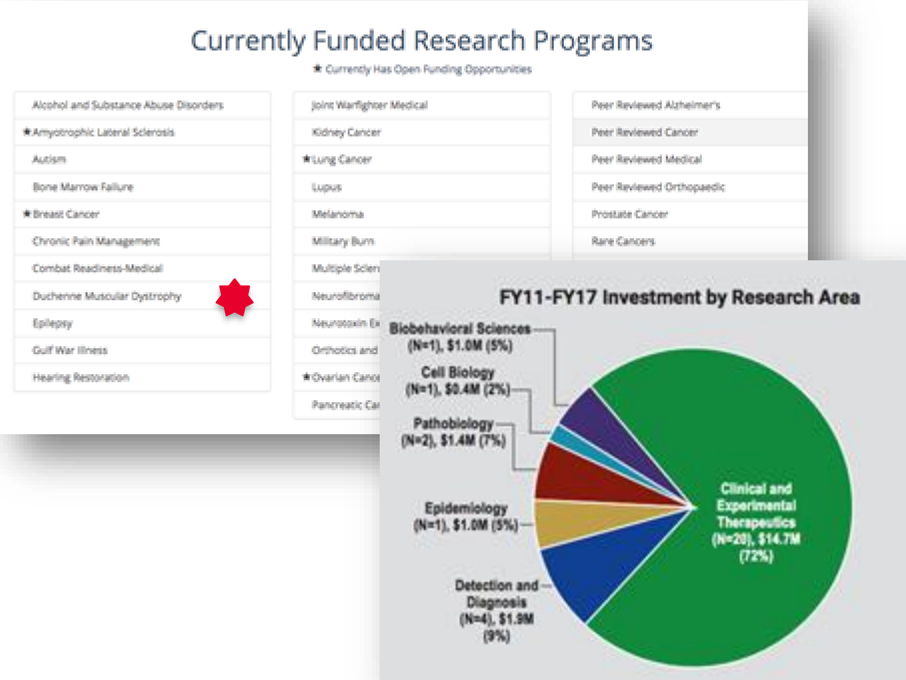
# Department of Defense

## Congressionally Directed Medical Research Program (CDMRP)

- **PPMD** partners with **The Foundation to Eradicate Duchenne** (FED) around advocacy related to this program.
- Peer reviewed and consumer reviewed grant process.

**Grants through the CDMRP have funded projects aimed at:**

- CRISPR
- Gene Therapy
- Steroids/Inflammation
- Outcome measures
- Biomarkers
- Cardiac



Currently Funded Research Programs

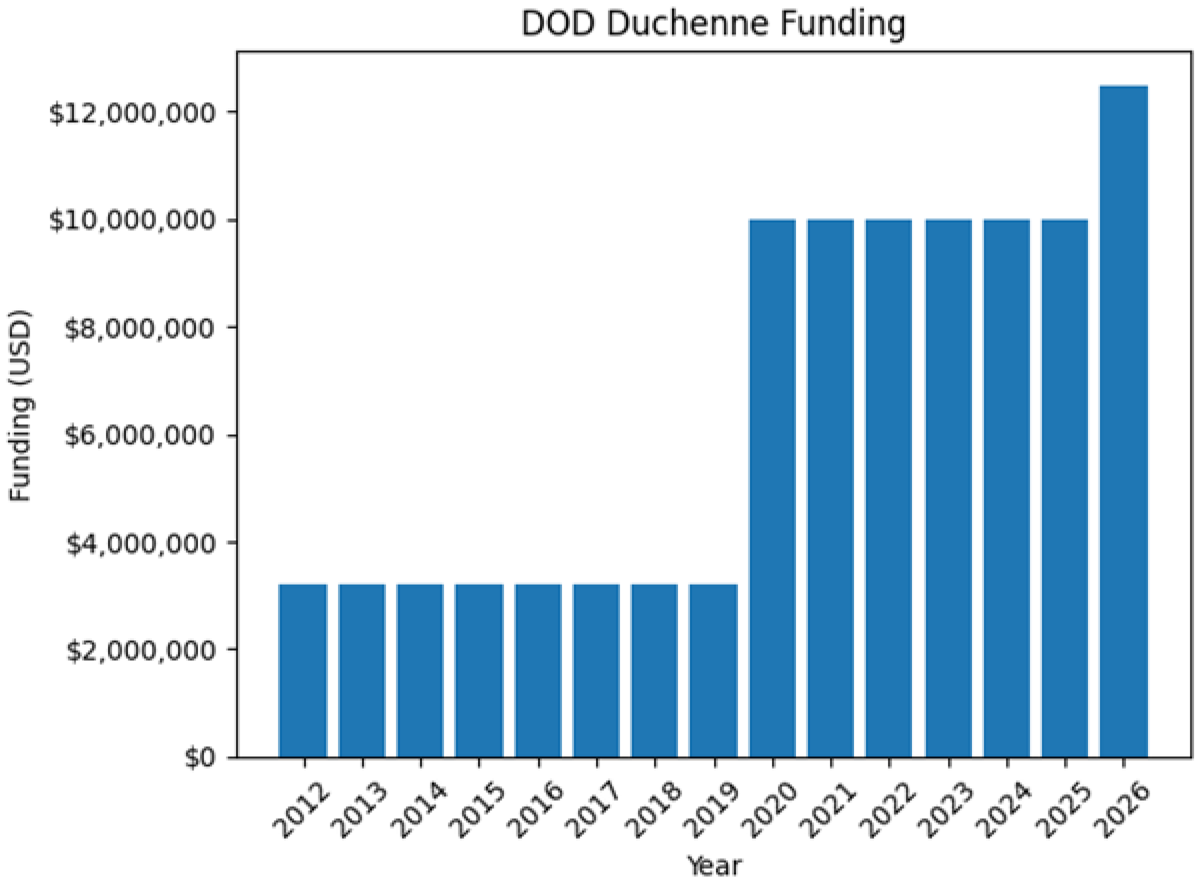
★ Currently Has Open Funding Opportunities

- Alcohol and Substance Abuse Disorders
- ★Amyotrophic Lateral Sclerosis
- Autism
- Bone Marrow Failure
- ★Breast Cancer
- Chronic Pain Management
- Combat Readiness-Medical
- Duchenne Muscular Dystrophy
- Epilepsy
- Gulf War Illness
- Hearing Restoration

- Joint Warfighter Medical
- Kidney Cancer
- ★Lung Cancer
- Lupus
- Melanoma
- Military Burn
- Multiple Scler
- Neurofibroma
- Neurotoxin E
- Orthotics and
- ★Ovarian Canc
- Pancreatic Ca

- Peer Reviewed Alzheimer's
- Peer Reviewed Cancer
- Peer Reviewed Medical
- Peer Reviewed Orthopaedic
- Prostate Cancer
- Rare Cancers

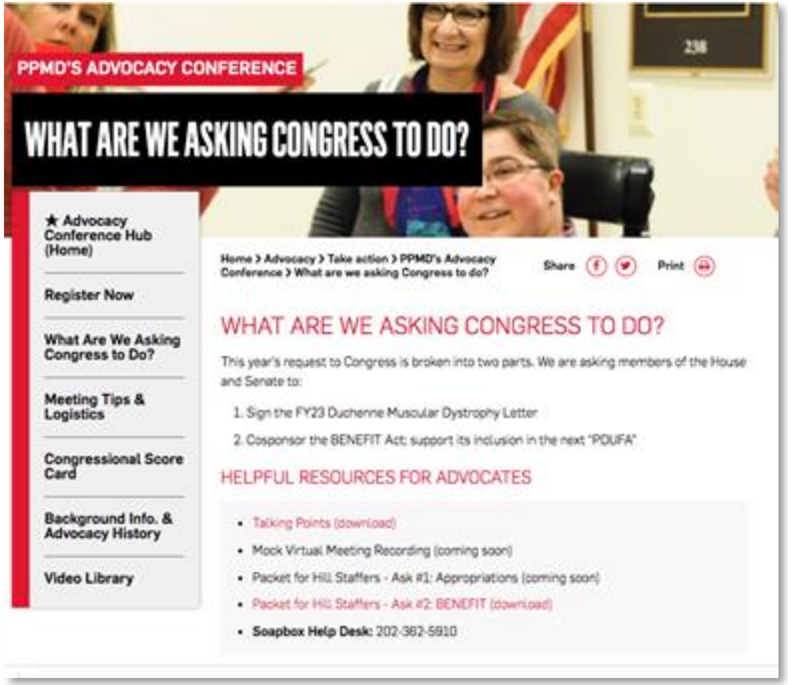
FY11-FY17 Investment by Research Area


DOD Duchenne Funding
$12,000,000
$10,000,000
$8,000,000
$6,000,000
$4,000,000
$2,000,000
$0
Funding (USD)
2012
2013
2014
2015
2016
2017
2018
2019
2020
2021
2022
2023
2024
2025
2026
Year

# Why the DOD?

# This didn't happen by accident

- CDMRP is funded through congress
- It exists because advocates pushed for it
- Every dollar is a result of community action



WHAT ARE WE ASKING CONGRESS TO DO?

This year's request to Congress is broken into two parts. We are asking members of the House and Senate to:

1. Sign the FY23 Duchenne Muscular Dystrophy Letter
2. Cosponsor the BENEFIT Act; support its inclusion in the next "PDUFA"

HELPFUL RESOURCES FOR ADVOCATES

- Talking Points (download)
- Mock Virtual Meeting Recording (coming soon)
- Packet for Hill Staffers - Ask #1: Appropriations (coming soon)
- Packet for Hill Staffers - Ask #2: BENEFIT (download)
- **Soapbox Help Desk:** 202-382-5910

# How advocacy turns into research funding

Advocacy Conference

↓

Capitol Hill Meetings + Calls + Emails

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Congressional Support

↓

Appropriations

↓

CDMRP Funding

↓

Duchenne Research

# What makes CDMRP Different

CDMRP
Department of Defense

- High-risk, high-reward
- Patient voice in review
- Funds the full pipeline

# Success story - Vamorolone

**From idea → to FDA-approved therapy**

- Novel **dissociative steroid** designed to separate efficacy from side effects
- Early research supported through CDMRP-funded work
- Advanced through clinical trials with strong community engagement
- **FDA approved in 2023** for Duchenne

# Other Success Stories

- Early work on **antisense oligonucleotides**
- AAV vector optimization
- immune response research
- delivery and dosing strategies
- Development of:
  - MRI-based muscle assessments
  - quantitative outcome measures
- Baby Duchenne
- ACTION/Cardiac
- Steroid replacement therapies
- GLP-1 Study

# None of this would be possible without our Advocates

If you are interested in getting involved in Advocacy – email Lauren Stanford at Lauren@parentprojectmd.org or Brian Denger at Brian@parentprojectmd.org

# Thank you!