

2025 Current Fiscal Year Report: Muscular Dystrophy Coordinating Committee

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1. Department or Agency

Department of Health and Human Services

2. Fiscal Year

2025

3. Committee or Subcommittee

Muscular Dystrophy Coordinating Committee

3b. GSA Committee No.

13889

4. Is this New During Fiscal Year? 5. Current Charter 6. Expected Renewal Date 7. Expected Term Date

No 08/15/2024 08/15/2026

8a. Was Terminated During Fiscal Year?

No

8b. Specific Termination Authority

8c. Actual Term Date

9. Agency Recommendation for Next Fiscal Year

Continue

10a. Legislation Req to Terminate?

Not Applicable

10b. Legislation Pending?

Not Applicable

11. Establishment Authority Statutory (Congress Created)

12. Specific Establishment Authority

42 USC 283g, section 404E

13. Effective Date

12/18/2001

14. Committee Type

Continuing

14c. Presidential?

No

15. Description of Committee Scientific Technical Program Advisory Board

16a. Total Number of Reports

No Reports for this Fiscal Year

17a. Open

1 17b. Closed 0 17c. Partially Closed 0 Other Activities 0 17d. Total 1

Meetings and Dates

Purpose

Start

End

The purpose of this meeting is to bring together committee members, representing government agencies, patient advocacy groups, other voluntary health organizations and people with lived experience to discuss topics of interest to the muscular dystrophy communities and renewal of the committee's strategic plan, the Action Plan for the Muscular Dystrophies.

11/22/2024 - 11/22/2024

Number of Committee Meetings Listed: 1

	Current FY	Next FY
18a(1). Personnel Pmts to Non-Federal Members	\$800.00	\$0.00
18a(2). Personnel Pmts to Federal Members	\$10,369.00	\$0.00
18a(3). Personnel Pmts to Federal Staff	\$0.00	\$0.00
18a(4). Personnel Pmts to Non-Member Consultants	\$0.00	\$0.00
18b(1). Travel and Per Diem to Non-Federal Members	\$0.00	\$0.00
18b(2). Travel and Per Diem to Federal Members	\$0.00	\$0.00
18b(3). Travel and Per Diem to Federal Staff	\$0.00	\$0.00
18b(4). Travel and Per Diem to Non-member Consultants	\$0.00	\$0.00
18c. Administrative Costs (FRNs, contractor support, In-person/hybrid/virtual meetings)	\$0.00	\$0.00
18d. Other (all other funds not captured by any other cost category)	\$23,955.00	\$0.00
18e. Total Costs	\$35,124.00	\$0.00
19. Federal Staff Support Years (FTE)	0.00	0.00

20a. How does the Committee accomplish its purpose?

The Muscular Dystrophy Coordinating Committee (MDCC) coordinates research activities across the National Institutes of Health, with other Federal health programs and voluntary health organizations, and activities relating to the various forms of muscular dystrophy, including Duchenne, myotonic, fasioscapulohumeral muscular dystrophy. The Committee has developed a plan for conducting and supporting research and education on muscular dystrophy through the national research institutes and other member organizations of the MDCC. The Committee will determine strategies for implementation of the plan, and will periodically review and revise the plan.

20b. How does the Committee balance its membership?

The Muscular Dystrophy Coordinating Committee consists of 18 members, including the Chair. Two-thirds of the members represent governmental agencies, including the directors or their designees of four of the national research institutes that support muscular dystrophy research, representatives of other Federal departments and agencies whose programs support research on the muscular dystrophies or provide services to people with these diseases including the Centers for Disease Control and Prevention, the Health Resources and Services Administration, the Food and Drug Administration, the Administration for Community Living, the Social Security Administration, the Centers for Medicare and Medicaid Services, the Department of Defense, and the Department of Education. One-third of the members are public members, including representatives from voluntary health organizations.

20c. How frequent and relevant are the Committee Meetings?

As described in the MD CARE Act (P.L. 107-84), the Muscular Dystrophy Coordinating Committee meets at least twice each year. The committee met two times during this reporting period. The committee met in December 2023 and March 2024. Meeting topics are selected with input from Committee members and are chosen for their relevance to the patient and research communities.

20d. Why can't the advice or information this committee provides be obtained elsewhere?

The Muscular Dystrophy Coordinating Committee is composed of both representatives of Governmental agencies and public members who have developed a plan for conducting and supporting research and education on muscular dystrophy. The formation of the Committee was mandated in the MD-CARE Act (P.L. 107-84). The Committee will periodically review and revise the plan and will discuss ways to further coordinate activities across the NIH, with other Federal health programs and voluntary health organizations relating to the muscular dystrophies. There are no other committees with the representation and experience of the MDCC.

20e. Why is it necessary to close and/or partially closed committee meetings?

N/A

21. Remarks

This committee did not produce any public reports during the fiscal year. Members: Gustavo Dziewczapolski, Emily Freilich, Walter Koroshetz, Daniel Perez, Natalie Street, and Eric Wang were on administrative extensions during the reporting

period. One member is no longer on the committee: Diana Bianchi represented the Eunice Kennedy Shriver National Institute of Child Health & Human Development as prescribed by the Charter. There are four vacancies, which explains the discrepancy in the Charter membership requirements.

Designated Federal Officer

Glen Nuckolls Program Director, Extramural Research Program

Committee Members	Start	End	Occupation	Member Designation
Bianchi, Diana	04/27/2018	12/31/2024	DIRECTOR, EUNICE KENNEDY SHRIVER, NATIONAL INSTITUTE OF CHILD HEALTH AND HUMAN DEVELOPMENT, NATIONAL INSTITUTES OF HEALTH	Regular Government Employee (RGE) Member
Criswell, Lindsey	07/07/2023	12/31/2024	Director, National Institute of Arthritis and Musculoskeletal and Skin Diseases (NIAMS), National Institutes of Health	Regular Government Employee (RGE) Member
Dziewczapolski, Gustavo	10/28/2018	12/31/2024	SCIENTIFIC DIRECTOR, CURE MD	Special Government Employee (SGE) Member
Freilich, Emily	07/28/2021	12/31/2024	Clinical Team Lead, Division of Neurology 1, Federal Drug Administration	Regular Government Employee (RGE) Member
Goldstein, Michael	07/21/2023	12/31/2024	Director, Office of Medical Policy, Social Security Administration	Regular Government Employee (RGE) Member
Keehn, Alisha	08/01/2023	12/31/2024	Genetic Services Branch Chief, Health Resources and Services Administration	Regular Government Employee (RGE) Member

Koroshetz, Walter	05/31/2018	12/31/2024	Director, National Institute of Neurological Disorders and Stroke	Regular Government Employee (RGE) Member
Lusane, Yolanda	01/01/2025	12/31/2027	Department of Education	Regular Government Employee (RGE) Member
McGuire, Marielena	05/22/2019	12/31/2024	Health Science Program Manager, Department of Defense	Regular Government Employee (RGE) Member
Perez, Daniel	05/14/2018	12/31/2024	President and Chief Executive Officer, Faciocapulohumeral Muscular Dystrophy Society, Inc	Special Government Employee (SGE) Member
Street, Natalie	05/17/2018	12/31/2024	HEALTH SCIENTIST,NATIONAL CENTER ON BIRTH DEFECTS AND DEVELOPMENTAL DISABILITIES CENTERS FOR DISEASE CONTROL AND PREVENTION	Regular Government Employee (RGE) Member
Wang, Eric	11/19/2023	12/31/2024	Associate Professor, Department of Molecular Genetics and Microbiology, University of Florida	Special Government Employee (SGE) Member

Number of Committee Members Listed: 12

Narrative Description

The formation of the Muscular Dystrophy Coordinating Committee (MDCC) was mandated by the MD-CARE Act (P.L. 107-84). The purpose of the MDCC is to coordinate research activities across the National Institutes of Health (NIH) and with other Federal health programs and activities relating to the various forms of muscular dystrophy, including Duchenne, myotonic, facioscapulohumeral muscular dystrophy and other forms of muscular dystrophy. As required by law, the function of the MDCC is to develop a plan for conducting and supporting research and education on muscular dystrophy through the national research institutes and periodically review and revise the plan. The MDCC has developed a research and education plan for the muscular dystrophies for NIH. This plan

lays out research goals for advancing research and treatments for the muscular dystrophies. The MDCC also has developed an Action Plan that identified key activities to be undertaken by the broader muscular dystrophy community (governmental organizations, advocacy groups, and academic and corporate investigators). This Action Plan is used as a means to identify and coordinate key activities.

What are the most significant program outcomes associated with this committee?

Checked if
Applies

Improvements to health or safety	<input checked="" type="checkbox"/>
Trust in government	<input checked="" type="checkbox"/>
Major policy changes	<input type="checkbox"/>
Advance in scientific research	<input checked="" type="checkbox"/>
Effective grant making	<input type="checkbox"/>
Improved service delivery	<input type="checkbox"/>
Increased customer satisfaction	<input type="checkbox"/>
Implementation of laws or regulatory requirements	<input checked="" type="checkbox"/>
Other	<input type="checkbox"/>

Outcome Comments

NA

What are the cost savings associated with this committee?

Checked if Applies

None	<input checked="" type="checkbox"/>
Unable to Determine	<input type="checkbox"/>
Under \$100,000	<input type="checkbox"/>
\$100,000 - \$500,000	<input type="checkbox"/>
\$500,001 - \$1,000,000	<input type="checkbox"/>
\$1,000,001 - \$5,000,000	<input type="checkbox"/>
\$5,000,001 - \$10,000,000	<input type="checkbox"/>
Over \$10,000,000	<input type="checkbox"/>
Cost Savings Other	<input type="checkbox"/>

Cost Savings Comments

Basic and clinical research accomplishments, as supported by MDCC member organizations, often take many years to unfold into new diagnostic tests and new ways to treat and prevent diseases. Coordinated efforts by the committee allow for screening and prevention of overlapping support for research projects.

What is the approximate Number of recommendations produced by this committee for the life of the committee?

2

Number of Recommendations Comments

The development of additional recommendations is in progress. During the November 2024 meeting of the MDCC, the committee discussed recommendations of draft priorities for renewal of the Action Plan for the Muscular Dystrophies (last renewed in 2015; available at mdcc.nih.gov). Four working groups of experts were organized that each included researchers, people with lived experience and representatives from patient advocacy groups. These working groups on 1) disease mechanisms, 2) preclinical translation, 3) clinical studies and standards of care, and 4) thriving with muscular dystrophy met periodically during the summer and fall to develop broad priorities for research and strategies to reduce obstacles to care and services for those living with muscular dystrophies. The Co-Chairs from these working groups presented their recommendations to the MDCC during the November meeting. We will seek additional input from the public on these draft recommendations and the MDCC will consider approving them to be included in the 2025 renewal of the committee's Action Plan.

What is the approximate Percentage of these recommendations that have been or will be Fully implemented by the agency?

70%

% of Recommendations Fully Implemented Comments

The Action Plan describes various research objectives that will take decades or longer to complete. NIH is currently supporting, and will continue to support, research grants that address approximately 70% of the topics in the Action Plan. Approximately 30% of the Action Plan relates to topics that are beyond the mission of the NIH, but relevant to other federal agency and voluntary group members of the MDCC. Approximately 60% of the objectives of the Action Plan have been the primary topics of funded NIH grants. Overall, approximately 70% of the objectives of the Action Plan have been fully implemented by support from NIH, other funding organizations, other Federal agencies and patient

advocacy organization members of MDCC as well as for-profit organizations not part of MDCC.

What is the approximate Percentage of these recommendations that have been or will be Partially implemented by the agency?

30%

% of Recommendations Partially Implemented Comments

Funding opportunities are active at NIH and other public and private MDCC member organizations that can be used to support many of the objectives of the Action Plan that are not yet fully implemented. These objectives are considered partially implemented if funding is available or if research has begun but goals have not yet been achieved.

Does the agency provide the committee with feedback regarding actions taken to implement recommendations or advice offered?

Yes ☒ No ☐ Not Applicable ☐

Agency Feedback Comments

Information is provided to the public at each meeting. The public can view information related to the Committee through the committee's official website.

What other actions has the agency taken as a result of the committee's advice or recommendation?

Checked if Applies

Reorganized Priorities	<input checked="" type="checkbox"/>
Reallocated resources	<input type="checkbox"/>
Issued new regulation	<input type="checkbox"/>
Proposed legislation	<input type="checkbox"/>
Approved grants or other payments	<input type="checkbox"/>
Other	<input type="checkbox"/>

Action Comments

NA

Is the Committee engaged in the review of applications for grants?

No

Grant Review Comments

NA

How is access provided to the information for the Committee's documentation?

Checked if Applies

Contact DFO	<input checked="" type="checkbox"/>
Online Agency Web Site	<input checked="" type="checkbox"/>
Online Committee Web Site	<input checked="" type="checkbox"/>
Online GSA FACA Web Site	<input checked="" type="checkbox"/>
Publications	<input type="checkbox"/>
Other	<input type="checkbox"/>

Access Comments

The MDCC website is located at: <https://mdcc.nih.gov/>. The website contains information about Committee membership, past and upcoming meetings, and contains a link to the Research and Education Plan for MD.