

**The 2015 Muscular Dystrophy Coordinating Committee (MDCC)
Action Plan for the Muscular Dystrophies
(The MDCC 2015 Action Plan)**

What is the MDCC?

The MDCC coordinates muscular dystrophy activities across the National Institutes of Health (NIH), with other Federal agencies and muscular dystrophy patient organizations. Committee meetings, held twice a year, are open to the public and are webcast for those who cannot attend in person. Two-thirds of the members represent government agencies; one-third represent the public, including patients and their families. The MDCC membership represents a broad range of expertise and perspectives on many forms of muscular dystrophy and the issues facing the researchers, patients, their families, and health care providers. A list of current members are available at <https://www.ninds.nih.gov/about-ninds/what-we-do/strategic-plans-evaluations/strategic-plans/muscular-dystrophy-coordinating-committee-action-plan-muscular-dystrophies>

What is the MDCC 2015 Action Plan?

The *MDCC 2015 Action Plan* (https://www.ninds.nih.gov/sites/default/files/documents/2015_MDCC_action_plan_508c.pdf), like the 2005 plan before it, outlines priority areas for improving treatments and reducing the personal and societal impacts of all types of muscular dystrophy. It reflects the deliberations of basic, translational, and clinical researchers who were nominated by the federal and public members of the MDCC; feedback from patient advocacy groups and people affected by the muscular dystrophies; and participation by the federal agencies represented on the MDCC. The MDCC approved the final plan in November 2015. Because the plan includes recommendations for the entire muscular dystrophy community, most objectives are relevant to multiple forms of muscular dystrophy. Communities with targeted interests are encouraged to use the *MDCC 2015 Action Plan* as a resource for developing or updating their own strategic plans.

What priority areas are covered by the MDCC 2015 Action Plan?

The *MDCC 2015 Action Plan's* 81 objectives can be summarized in five priority areas:

- **Understanding Causes**—Promote research into the causes of muscular dystrophies and of patient-to-patient variations within a single disease to advance the development of diagnostic strategies and treatment approaches.
- **Screening and Diagnosis**—Advance the implementation of comprehensive screening and other activities that will lead to rapid diagnosis using minimally-invasive methods, and use the resulting information and samples to optimize clinical care and accelerate research.
- **Developing Treatments**—Expand and intensify the development and identification of treatments that, alone or in combination, significantly slow or reverse disease progression across all affected organ systems.

- **Preparing for Clinical Trials**—Accelerate the development and validation of tools for conducting rigorous clinical trials that require the fewest participants for the shortest duration to achieve conclusive results. Trials should measure disease-related changes important to patients and should focus on the most promising therapeutic candidates.
- **Providing Care, Management and Access to Services**—Advance efforts to evaluate and optimize the clinical care, self-management, and access to services for people who have muscular dystrophy.

Who is responsible for implementing the MDCC 2015 Action Plan?

All stakeholders—including academic researchers, companies, government agencies, patient advocacy groups, and patients and their families—have a shared responsibility for achieving the plan’s objectives, which are ambitious and some may take a decade or more to achieve. Adequate solutions to the priority areas covered by the plan will require a considerable level of cooperation among stakeholders and collaborations with new partners. The *MDCC 2015 Action Plan* provides a roadmap for these collaborations.

How does the MDCC 2015 Action Plan reflect advances in the field since the 2005 Plan?

Since the development of the *2005 MDCC Action Plan for the Muscular Dystrophies*, the field has seen tremendous progress toward understanding the mechanisms that cause muscular dystrophies and their symptoms, the initiation of clinical trials of potential therapies, improved clinical management, and better overall health and longer lives for people who have muscular dystrophies. The *MDCC 2015 Action Plan* aims to continue to build upon these efforts through:

- A deeper understanding of disease mechanisms to aid in the identification of potential new therapies and ways to predict these compounds’ effects on people;
- Better aggregation of molecular, tissue, and patient data resulting in earlier and more reliable diagnoses and efficiently designed clinical trials;
- Improved vetting of candidate therapeutics to better prioritize which should proceed to clinical testing; and
- Increased efforts to improve the quality of life, education, and employment opportunities of people who are living with muscular dystrophies.

What’s next?

At the November 2015 meeting, the MDCC agreed to begin collecting information about member agencies’ and organizations’ activities related to the priority areas in the *MDCC 2015 Action Plan*. However, many other groups not directly represented on the MDCC also support activities that advance the objectives outlined in the plan. The MDCC is considering strategies for learning about these efforts so that the Committee can assess more accurately how the *MDCC 2015 Action Plan’s* priorities are being addressed. The MDCC encourages all stakeholders to notify their research communities about the important priorities for the field outlined in the *Action Plan* and to use the plan to help explore opportunities for strategic collaborations with other stakeholders to benefit the muscular

dystrophy community. For more information or to share information about how your organization is supporting *Action Plan* objectives, please contact Dr. Glen Nuckolls, MDCC Executive Secretary at nuckollg@ninds.nih.gov.