



Myotonic Dystrophy Foundation

**Strategic Plan
2021-2024**

Approved by MDF Board of Directors
December 14, 2021

Introduction



Our History

The Myotonic Dystrophy Foundation (MDF) was founded in 2007 to improve the quality of life of people living with myotonic dystrophy (DM) and accelerate the search for therapies and a cure.

Over the past 14 years, MDF has invested over \$25 million in research and programs to become the largest DM-focused patient advocacy organization in the world, serving tens of thousands of individuals and families across the United States and 139 countries.

MDF's programs fund critical research, provide comprehensive resources and support to people living with the disease and their families, and advocate for government agencies to enhance the drug development pipeline, increase research funding, and improve patient services.

Recent achievements related to patient care, advocacy, and research toward a cure include, but are not limited to:

- **More than 15,000 DM patient and family members are served** by support programs and resources every year
- Improving care for affected individuals by **publishing clinical care recommendations** with an international network of medical experts
- Hosting the **largest annual DM-focused conference in the world**, delivering education and support programs, convening stakeholders and connecting researchers, industry professionals, and community members

- Over the last 5 years, MDF invested more than **\$5 million in a drug development acceleration** effort including 15 major initiatives designed to attract more industry investment, lower barriers to therapy development, and expand the amount of data available to drive discovery
- Helping attract more than **45 companies and institutions** into DM drug development with the expectation of several new therapies entering clinical trials in 2022
- More than **30 DM research fellows** have been funded leading to over **70 peer-reviewed publications** now advancing DM academic knowledge and research
- Maintaining one of the **largest DM patient registries in the world** to collect data needed for better disease understanding and ensure that the community is organized and trial-ready
- Advocate for **additional DM funding** from the US Department of Defense, which has led to nearly \$8 million in new government funding since 2018.

In 2021, MDF launched a strategic planning process to envision the near-term future of the organization over the next three years. This document summarizes the results of that process.



Our Challenge

Myotonic dystrophy (DM) affects every aspect of life for individuals living with the disease as well as their family and loved ones. DM is an inherited disorder that can appear at any age and manifests differently in every person. There is currently no treatment or cure.

While the end objective – treatment and a cure – is clear, the DM community faces a series of scaffolded challenges in getting there.

As a lesser-known rare disease, progress toward treatment development or a cure for DM is hampered by significant under-diagnosis. While research indicates that as many as 1 in 2,100 individuals are affected by myotonic dystrophy or at risk of passing the disease to the next generation, only a small fraction of those individuals are diagnosed, and an even smaller number of those diagnosed have access to the care and resources needed to experience a high quality of life. In the United States, this means that while there may be more than 150,000 individuals with the disease, there are currently fewer than 2,500 registered with the Myotonic Dystrophy Family Registry.

Under-diagnosis has implications for the development of treatments and, ultimately, a cure for DM. Since the population of individuals affected appears to be smaller than prevalence data suggests, due to under- and mis-diagnoses, it is harder to make the case for the pharmaceutical industry to develop treatments or for researchers to conduct new research. Under-diagnosis is particularly acute for DM2, and consequently those affected have access to even fewer resources and support.

For those who are diagnosed with DM, finding access to appropriate care from clinicians and healthcare professionals can be particularly challenging. It can be difficult to access support and resources relevant to the challenges and lived experiences of people affected by the disease, including support networks of other affected individuals and families. This challenge is further compounded by systemic level differences in which groups have appropriate access to care and resources based on factors such as race, class, gender, and geography. Further, symptoms of the disease may make it challenging for some affected individuals to perform daily tasks, which presents additional barriers to accessing resources and care.

The complicated nature of the disease makes supporting patients and providing clear treatment guidelines for medical professionals more difficult. As DM can impact nearly every system of the body in a variety of ways, educating doctors and creating complete guidelines is arduous. It also makes it difficult to measure the impact of treatments or isolate impacts in drug development and clinical trials. Finally, other muscular dystrophies and rare diseases more widely recognized and better understood than DM receive larger amounts of funding. Access to funding has significant implications for disease research and drug development.

Methodology

This work is the result of a strategic planning process facilitated in spring of 2021 by Third Plateau, a social impact strategy firm. This plan reflects the collective thinking and commitment of representatives from across MDF’s ecosystem. The process was guided by an 11-person Strategic Planning Steering Committee comprising MDF’s CEO and members of the MDF community, which included individuals and families affected by DM, board members, representatives of the scientific and research community, clinicians, and other organizational partners and advocates.¹ MDF’s Board of Directors and staff members also engaged in the planning process. In partnership with the Strategic Planning Steering Committee, Board of Directors, and MDF staff, Third Plateau engaged in a three-phase planning process.²

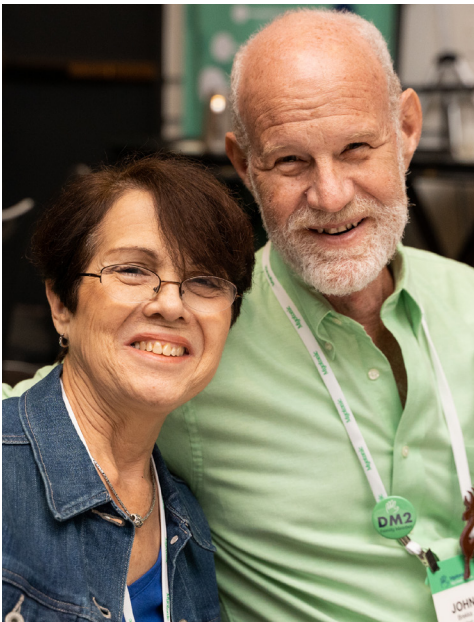
The “**Learning**” phase focused on understanding MDF’s current state. Through primary and secondary research, the planning team sought to: identify MDF’s strengths, weaknesses, opportunities, and threats; learn from the field about how MDF might effectively organize our work and engage our community; and directly engage as many members of the community as possible to understand their needs and challenges and to consider their perspectives and experiences in shaping our future. In this phase, more than 800 participants engaged through interviews, focus groups, and a community survey. **Figure 1** below overviews the research components, outlining their various purposes, participants, and methods.

Figure 1. Research Methods Overview

RESEARCH DELIVERABLE	PURPOSE	RESEARCH PARTICIPANTS	ANALYSIS METHODS
<i>Field level:</i> Research Memo	To learn lessons that MDF might apply to its own work from comparable organizations by examining other rare disease organizations and their size, structure, and approaches to engaging their communities and experts.	<ul style="list-style-type: none"> • 3 in-depth interviews with leaders of rare disease organizations • Online research of 16 rare disease organizations 	<ul style="list-style-type: none"> • Researched the landscape of rare disease organizations relevant to MDF and spoke to leaders at 3 comparable organizations.
<i>Community level:</i> Survey Findings	To understand the needs and challenges of the community as well as potential future areas of focus.	<ul style="list-style-type: none"> • 717 unique survey responses 	<ul style="list-style-type: none"> • Created a 46-question survey in consultation with the MDF team and trusted advisors. • Analyzed the results using both quantitative and qualitative analysis software.
<i>Organizational level:</i> SWOT Analysis	To understand perceptions about the current state of MDF, categorized as present day strengths, weaknesses, opportunities, and threats.	<ul style="list-style-type: none"> • 36 individual interviews • 18 focus group conversations, consisting of 71 individuals 	<ul style="list-style-type: none"> • Qualitatively coded responses, grouping them into themes. • Synthesized themes in order of salience, prioritized based on how many stakeholders referenced them and their potential relevance for the strategic planning process.

¹ A full list of Strategic Planning Steering Committee members is provided in Appendix A.

² A full list of Board Members and a list MDF staff are provided in Appendices B and C.



The second phase, **“Ideation,”** focused on defining MDF’s desired future state. Third Plateau facilitated a series of planning sessions with the Strategic Planning Steering Committee, Board of Directors, and staff to use the research findings to refine MDF’s vision, mission, and theory of change. The group also worked to address how to close the gap between the organization’s current state and desired future, participating in working sessions and strategy brainstorming to set three-year goals, strategies, and tactics.

The third and final phase, **“Iterative Design,”** focused on drafting and refining the plan, as well as preparing for the implementation of the plan. Key considerations included: a feasible implementation timeline, financial implications, and critical milestones and metrics. Throughout the process the strategic plan was shared with the Strategic Planning Steering Committee and select stakeholders for feedback.

Our Purpose

OUR VISION:

We envision a world with treatments and a cure for myotonic dystrophy.

OUR MISSION:

The mission of the Myotonic Dystrophy Foundation is Community, Care, and a Cure.

We support and connect the myotonic dystrophy community

We provide resources and advocate for care

We accelerate research toward treatments and a cure



Our Core Values

Our core values guide our approach to pursuing our vision and mission. The values are foundational to MDF's work and reflect the characteristics and behaviors to which we aspire, both as an organization and across our broader community.

COMMUNITY

Our community is our greatest asset. We believe that people living with DM are the experts of their own disease and experience. We prioritize cultivating our community as an engaged extended family of individuals living with DM, healthcare professionals, researchers, and other organizational partners and advocates.

EMPATHY

We approach our work and our relationships with empathy, consistently seeking to understand others' points of view and unique lived experiences, both to build trust across our community and to inform our work.

HOPE

We tackle challenges and uncertainty with hopefulness and optimism. We work towards significant outcomes that improve lives, with the steadfast belief that there will be effective treatments and a cure.

COLLABORATION

We value collaboration. We recognize that it is through shared commitment and effective team work with many partners across our network that we will make meaningful progress.

KNOWLEDGE

We are committed to ongoing learning, knowledge sharing, and continuous improvement. We recognize our role in building and sharing knowledge that will improve the quality of life for our community and accelerate progress toward treatments and a cure.

URGENCY

We operate with persistent focus and determination to address the pressing needs of our community. We effectively prioritize our work and actions to ensure we have the most significant impact toward our vision.

2021 – 2024 Strategic Goals



To advance toward our mission and vision, Myotonic Dystrophy Foundation will prioritize four key Goals over the next three years. We will pursue targeted strategies and tactics associated with each Goal area summarized below.

GOAL 1. COMMUNITY: Strengthen our community.

Across the next three years, we will prioritize growing and strengthening our community, which includes individuals living with DM1 (congenital, juvenile-onset, and adult), and DM2, and their families and caregivers. Based on what we know about the prevalence of myotonic dystrophy, there are many individuals and families who are affected that are not yet engaged with MDF or our current programs and resources.

Further, we recognize that racism and social inequity are entrenched in many of our systems in the United States, which means that certain marginalized and traditionally underserved populations may be especially under- and misdiagnosed, and experience additional barriers to quality healthcare, to information and technology, and many other resources necessary to experience high-quality of life. MDF recognizes these setbacks can be devastating and will continue to work to eliminate those barriers for all.

By effectively reaching and engaging as many individuals living with DM as possible and continuing to improve the quality and range of resources and programs for the community, we aim to build connection, lessen the burdensome impacts of the disease, and cultivate hope for treatments that improve quality of life.

GOAL 2. CARE: Improve access to effective healthcare that meets the needs of individuals and their families.

Our aim is to ensure that individuals who are experiencing symptoms of DM are properly diagnosed and have access to care that meets their needs, improves wellbeing, and instills hope. In order to meet this aim we will take a multi-pronged approach to build awareness around myotonic dystrophy among the healthcare community and general public and provide high-quality resources that support individuals and families as well as practicing clinicians and healthcare professionals.

We will continue to educate the healthcare community, including clinicians, insurance companies, and other healthcare providers, advocating for improved access to care and diagnostic tools.

2021 – 2024 Strategic Goals



GOAL 3. CURE: Eliminate barriers to accelerate drug development.

MDF can identify and eliminate barriers to developing treatments for myotonic dystrophy through acting as a trusted and credible liaison between industry and our community and advancing a policy and funding environment conducive to research.

MDF plays a crucial and facilitative role in connecting and promoting collaboration across industry, researchers, and regulating agencies, the community of individuals and families affected by the disease, and network of healthcare professionals. We are positioned to leverage our role in bringing together the broader eco-system to accelerate the development of drug treatments and a cure.

GOAL 4. ORGANIZATIONAL STRENGTH: Build a strong, sustainable organization.

We will prioritize strengthening and building the capacity of MDF to pursue our mission over the long-term. Specifically, we will focus on building a sustainable and adaptable organization that is equipped with the leadership, financial resources, and staffing necessary to meet the needs of our network in an enduring way.

We recognize that strong team culture is the bedrock for high-performing organizations and we remain committed to further developing and maintaining culture that prioritizes our people, listens and learns from stakeholders, and is committed to ongoing staff growth and development in line with our vision, mission, and values.



APPENDIX A. Strategic Planning Steering Committee Members

Kevin Brennan, Bluebird Strategies
Loraine Dressler, MDF Support Group Facilitator
Dr. Belen Esparis, University of Pennsylvania
John Fitzpatrick, MDF Board Member and Strategic Planning Chair
Sarah Howe, MBA, Marigold Foundation
Jeremy Kelly, MDF Board Chair
Dr. Leslie Krongold, MDF Support Group Facilitator
Tom McPeek, Board Member, MDF Support Group Facilitator
Tom Silk, MDF UK Board of Trustees, Chair
Dr. Tanya Stevenson, CEO MDF
Dr. Eric Wang, University of Florida



APPENDIX B. MDF Board of Directors (United States)

David Berman
Dr. John W. Day
John Fitzpatrick, Strategic Planning Committee Chair
Elizabeth Florence, Secretary
David Herbert, Treasurer
Jeremy Kelly, Chair and Lifetime Trustee
Tom McPeek, Support Group Facilitator
Martha Montag Brown, Vice Chair
Joel Revill
Dr. Charles Thornton

APPENDIX C. MDF Staff

Kate Beck, Special Projects Manager
Kleed Cumming, Communications and Technology Manager
Allison Formal, MBA, Senior Science Advisor
Mike Knaapen, Program Director
Sara Littlefield, Development Associate
Dr. Nadine Skinner, Research Coordinator
Dr. Tanya Stevenson, CEO



APPENDIX D. Theory of Change

MDF's theory of change connects our mission to our vision. It outlines the logic behind how the Foundation's mission and core activities will produce outputs and outcomes that will bring us closer to our envisioned future where no one is experiencing the negative effects of myotonic dystrophy. Please see the diagram below for a visual representation.

MDF takes a comprehensive and collaborative approach. Through direct services, education, and advocacy, we promote a vibrant and connected community, increase access to appropriate healthcare and crucial resources, and eliminate barriers to drug development. This approach leads to reduced time to diagnosis, improved clinical care, higher quality of life, advancements in research and approved drugs, and ultimately, a cure.





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**Myotonic
Dystrophy**
FOUNDATION

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