MDCC | Muscular Dystrophy Coordinating Committee

2025 MDCC Action Plan for the Muscular Dystrophies
Thriving with Muscular Dystrophy
Draft Priorities

Introduction

While there are many key reforms necessary to improve health outcomes, quality of life, and access to benefits and services for children and adults living with muscular dystrophy (MD), the three priorities established as being meaningful and attainable to support the ability for people to thrive with MD are:

Priority topic 1: Overcome obstacles to access benefits that would enable children and adults living with muscular dystrophies (MD) to realize their full potential as active, contributing and autonomous members of society:

Through advancements in research, quality of care, accessibility, and education, children and adults living with all types of MD are arguably better positioned for pursuing education, employment, and independent living than ever before. However, current disability programs and benefits policies may not be optimizing access to services and economic self-sufficient for people living with MD. This discouraging situation may continue unless reforms promoting independence and economic self-sufficiency for people living with MD, and all Americans with disabilities, are pursued.

Social Security Disability Insurance

Almost one-third of those who receive SSDI benefits for any disability/condition receive them only after appealing an initial denial.[1] Furthermore, the initial application process can take many months, especially in the case of a denial, and the appeals process often takes much longer, sometimes several years. Nearly half of those denied benefits eventually have their denial overturned while many others simply give up altogether. Furthermore, an Office of the Inspector General Report[2] in 2010 documented wide variance in the allowance rates for certain conditions across different SSA regional offices, which may suggest the need for improvements in the consistent application of medical eligibility criteria. Applicants often must employ lawyers to successfully obtain their deserved benefits. Even once the SSA has determined the individual is eligible, the beneficiary must wait 5-months to receive benefits. Programs such as the SSA Compassionate Allowances program, which streamlines this process for individuals with specific diagnoses, address some of these challenges but the threshold for condition addition precludes most muscular dystrophies from inclusion.

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[1] USAFACTS: What is the approval rate for Social Security Disability Benefits? Dec. 12, 2023. https://usafacts.org/data-projects/disability-benefit-process

[2] Office of the Inspector General, Social Security Administration, Audit Report. Aug. 2010; https://oig-files.ssa.gov/audits/full/A-07-09-19083.pdf

Approaches for consideration: shorten and streamline SSDI's application process; promote consistent application of eligibility requirements; shorten or eliminate the waiting period; expand and systematically update the SSA CAL list.

Supplemental Security Income

Similarly, over 70 percent of SSI applicants are deemed ineligible upon applying. Once receiving benefits, the ease at which individuals can lose benefits also undermines economic self-sufficiency. Individuals must limit their assets to under $2,000 and married couples must limit their assets to under $3,000 to maintain eligibility. These levels have not been updated since 1989. Moreover, SSI beneficiaries are eligible for Medicaid, often a lifeline for their health and independence. Some who have lost eligibility for SSI may be able to maintain their Medicaid eligibility, but current rules sharply reduce benefits as a beneficiary's earnings increase. Individuals cannot save for large expenses and cannot own many valuable items. This potentially traps individuals living with MDs in poverty. Furthermore, the asset limitations in SSI prevent many individuals living with MDs from marrying as doing so would elevate the couple's assets over $3,000. Rather than risking losing SSI and Medicaid, individuals living with MDs could possibly face a decision between marrying (or staying married) and maintaining benefits. **Approaches for consideration:** raising SSI asset limits; increase the limits annually; increase monthly benefits.

ABLE Accounts

ABLE accounts, tax-deferred accounts that allow individuals with certain functional disabilities to save money for disability-related expenses without losing access to public benefit programs, were created at least in part to address the economic stability challenges described above. However, eligibility for ABLE accounts is limited, including requiring the disability in question to have appeared before the age of 26 (soon to be age 46 starting in 2026), requiring disposable income to be available for depositing into the ABLE account, and then the use of those funds is limited to specific disability-related purposes. Despite current limitations, the ABLE accounts are the first federal program that disentangles a poverty threshold from disability for eligibility. **Approaches for consideration:** expand eligibility and usability for ABLE accounts.

While the SSDI, SSI, and ABLE programs can all be improved, the discussion should continue on system wide reforms that replace current approaches with a system that ensures maximum economic self-sufficiency at a reasonable standard of living for people living with MD. This could entail eliminating the inability-to-work requirement in favor of systemwide eligibility criteria designed to identify people with significant functional limitations[3] – including people living with MD. This would include a variety of programs, including key health and independent

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[3] Defined as "a significant deviation or loss in body physiology or structure, such as loss of sensation in extremities, visual or hearing loss, paralysis, or anxiety." International Classification of Functioning, Disability, and Health: Children & Youth Version: ICF-CY. World Health Organization, 2007.

living-fostering benefits, like personal assistive services. Although some medical criteria will be needed to determine who is eligible for any support from the system, the eligibility criteria should move toward a functional definition of disability.[4] Approaches for consideration: explore uniform criteria for eligibility across all programs that do not use an inability-to-work standard.

Complex and Uncoordinated Program Structures Exacerbate Access Barriers

The complexity and poor coordination of support systems for people with disabilities also undermines the living standard of the MD community. The many in-kind supports available to individuals and families within the MD community include health insurance benefits, personal care assistance, assistance with technology purchases, food, housing, transportation, education, and others—are administered by a variety of state and federal agencies and private organizations, each with its own rules, many of which are very complex. Substantial numbers of people living with MD receive cash benefits from programs other than SSDI and SSI, especially Temporary Assistance for Needy Families. Understanding, obtaining, and managing the various supports requires substantial effort. **Approaches for consideration:** explore coordination across Federal, state, and non-governmental financial and in-kind benefit programs that assist the MD community.

Health Insurance Coverage and Portability

According to the CDC's MD STARnet surveillance program, a small portion of people living with MD report being uninsured or self-pay, while most have private or public insurance and some have both[5].

State-specific Medicaid programs and variations in private insurance coverage can create significant barriers for patients living with MD who need to relocate or change jobs. Furthermore, many state Medicaid programs have extensive waiting lists for eligible individuals to obtain home and community-based services beyond simple home care through their Medicaid programs, with some individuals waiting years for assistance[6]. **Approaches for**

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[4] As recommended by others (e.g. Stapleton DC, O'Day BL, Livermore GA, Imparato AJ. Dismantling the poverty trap: disability policy for the twenty-first century. Milbank Q. 2006;84(4):701-32. doi: 10.1111/j.1468-0009.2006. 00465.x. PMID: 17096639; PMCID: PMC2690299.

[5] Wallace B, Smith KT, Thomas S, Conway KM, Westfield C, Andrews JG, Weinert RO, Do TQN, Street N; Muscular Dystrophy Surveillance, Tracking, and Research Network (MD STARnet). Characterization of individuals with selected muscular dystrophies from the expanded pilot of the Muscular Dystrophy Surveillance, Tracking and Research Network (MD STARnet) in the United States. Birth Defects Res. 2021 Apr 15;113(7):560-569. doi: 10.1002/bdr2.1764. Epub 2020 Jul 24. PMID: 32710484; PMCID: PMC8114775.

[6] Burns A, Mohamed M, O'Malley Watts M. "A Look at Waiting Lists for Medicaid Home and Community-Based Services from 2016 to 2023" Nov. 2023

consideration: Expand benefit coverage for individuals living with MD that remains portable across state lines as a critical step towards improving equitable access to clinical care, novel therapies, and key resources.

Priority topic 2: Improve equitable access to resources for transportation, housing, DME, caregiver support and community opportunity.

While recent improvements in clinical outcomes of people living with MD may reduce their burden of disease and strengthen their functional abilities at the individual level, significant barriers exist that prevent people living with MD from accessing resources that improve daily living. There have not been any widespread significant, and measurable gains in reducing barriers to equitable societal opportunities and resources over the last decade. These societal barriers serve to limit and restrict opportunities, impacting their ability to achieve their full potential, and the presence of these barriers places an unfair tax on those who must rely on care support to fulfill basic needs. **Approaches for consideration:** Explore a multifaceted approach to ensuring equitable access for individuals living with MD, including transportation, housing, durable medical equipment (DME), and caregiver support, as well as opportunities to develop autonomy, enhance social roles and relationships, and narrow the gap between cultural barriers.

Few of these challenges are limited to those with MD. Individuals living with a disability, particularly mobility limitations, experience inequities often regardless of their diagnosis. **Approaches for consideration:** partner with other disease and disability communities to address challenges.

Transportation

Accessible transportation is an important determinant of self-sufficiency, social participation and integration into the educational system and workforce. Yet people living with MD are frequently faced with elevator outages in subway stations, gaps in train platforms, few, if any, accessible taxis or rideshares, prohibitively expensive wheelchair vans, and no wheelchair spots on airplanes. **Approaches for consideration:** expand and improve reliability of public transportation options with power wheelchair accessibility and other accommodations for disabled travelers. Where public options are inadequate, strategies should be considered such as grants or subsidies for individuals living with MD to afford private transportation or ride-sharing services. Local volunteer driver programs should be developed or partnerships with ride-sharing companies to provide affordable, reliable, on-demand transportation. The MD community would benefit from partnering with other disability communities to pursue these goals.

Durable Medical Equipment (DME)

Access to DME is paramount to the health of those living with MDs, including power wheelchairs, specialized seating, breathing equipment, and specialized DME that improves daily living. Insurance providers need to be more aware of the needs of MD-affected individuals and their caregivers so the definition of "medically necessary" is more inclusive of those needs and can be flexible as needs change over time. **Approaches for consideration:** expand government and nonprofit programs that provide financial assistance and low-interest loans for purchasing or renting DME; provide better access to information about available DME options and resources through centralized information hubs and advocacy organizations; conduct additional research to assess the impact of DME availability on community activity, participation and quality of life to inform decision making by public and private organizations.

Housing

Affordable and accessible housing is a fundamental need and people living with MDs experience significant obstacles. **Approaches for consideration:** explore ways to expand the availability of affordable housing units and support initiatives that retrofit existing housing that meet a wide variety of accessibility needs. Enhancements are needed for financial assistance programs to increase funding for programs that assist with home modifications, such as ramps, widened doorways, and accessible bathrooms. Partnerships with local businesses and/or vocational schools would help to educate and outsource minor home remodeling projects that improve accessibility. Changes in regulations and incentives for builders to incorporate universal design principles in new housing projects could be considered.

Caregiver Support

Caregivers, including family members, friends and employed service providers greatly affect the quality of life of individuals with MDs. Additional training programs and resources for caregivers are needed to better understand evolving symptoms of MD and develop effective caregiving strategies. Caregiver health and wellbeing significantly contributes to the quality of the care they provide. **Approaches for consideration:** explore reimbursement of familial caregivers and funding for respite care programs that give primary caregivers regular breaks while ensuring quality care for their loved one/client

Community Opportunities

Participation in community activities and social engagement are vital for the quality of life of people living with MDs. **Approaches for consideration:** Create and promote additional community programs for all individuals with MD, such as adaptive sports, social events, and educational workshops; increase awareness and understanding about MD within communities to foster greater empathy and support; include the perspectives of people with lived experience in the planning and maintenance of public spaces and recreational venues to ensure that the facilities are accessible and welcoming to affected individuals, including proper wheelchair access and adaptive equipment. Examples of good practices include the design of playgrounds and parks allowing for improved integration of community members with disabilities; the World Health Organization has a social initiative to improve design of public areas to encourage physical activity for people with disabilities. Incentives for companies to install wheelchair accessible, upper arm ergometers or education for people at community centers and gyms to work with and monitor safety and make adaptive community gyms and athletic centers more widely available.

Cultural Competency and Sensitivity

People living with MDs represent the cultural diversity of the general population along with cultural effects of their condition. Cultural competency training is needed for healthcare providers, caregivers, and support staff to understand and respect diverse cultural practices and values related to different forms of MD. Healthcare providers, researchers, and MD support organizations should actively engage with community leaders, cultural institutions, and grassroots organizations representing individuals of diverse ethnic, racial, sex, gender and linguistic identities. By collaborating with respected figures such as religious leaders, community elders, and local advocacy groups, these stakeholders can gain valuable insights into cultural beliefs, practices, and barriers that may impact full participation within the community. This approach allows for the development of culturally tailored education materials, outreach programs, care strategies, and inclusive design spaces that resonate with specific communities, ultimately improving access to care, increasing research participation, and enhancing overall health and wellness outcomes for individuals with MD from diverse backgrounds.

Priority topic 3: Make changes in the healthcare ecosystem to improve timely genetic diagnosis and access to high-quality multi-disciplinary care that is optimized for the individual's age, gender, abilities and other personalized factors.

Significant enhancements are needed in the healthcare ecosystem to ensure timely genetic diagnosis and access to clinical care that maximizes the longevity and quality of life of individuals with muscular dystrophies.

The Cost of Delayed Diagnosis in Rare Disease: A Health Economic Study released in 2023 by the EveryLife Foundation and Optum Health found that the mean diagnostic odyssey was more than 6 years, included up to 17 medical providers, and included avoidable costs ranging from $86,000 to $517,000. With the advent of diagnostic and newborn screening technology, timely diagnosis prevents catastrophic, irreversible disease progression, reduces costs to the individual, family, and society, facilitates clinical trial participation, and opens the door to appropriate treatment and care when such options exist. The advent of disease modifying cell and gene therapies in MDs only increases the salience of near immediate treatment delivery upon diagnosis. Multiple strategies could be pursued.

Newborn Screening

While many diagnostic platforms exist to enable earlier detection, it is our state newborn screening (NBS) programs, collectively diagnosing approximately 12,500 infants each year in the U.S., that could facilitate early identification and provision of life-altering treatment for. Adding conditions to the Secretary of Health and Human Services' (HHS) Recommended Uniform Screening Panel (RUSP) is a lengthy process that includes an evidence-based review of available data and information. The initial identification of available information and data is part of the nomination process, which is often led by a patient advocacy organization. The Advisory Committee for Heritable Disorders in Newborns and Children (ACHDNC) reviews the evidence and provides recommendations to the Secretary of HHS. Currently, the ACHDNC can conduct evidence reviews for two nominated conditions per year. Of note, adding conditions to newborn screening programs is up to each individual state and due to a variety of factors can take many years from the time a condition is added to the RUSP for states to add a condition to their state panel. **Approaches for consideration:** explore options to improve support for state newborn screening programs to help states have the capacity to more quickly implement RUSP-approved conditions and reform the RUSP process to accommodate more nominations and reduce the burden on nominating patient advocacy organizations.

Note: Duchenne muscular dystrophy (DMD) has been nominated to the RUSP and is currently undergoing an evidence-based review by the ACHDNC; several states have already added DMD to their state panels.

Genetic Testing

Genetic testing, which can often be performed on locally collected blood samples or through other non-invasive means, can confirm MD diagnoses and identify specific gene mutations in patients of any age. This information is invaluable for guiding treatment decisions and identifying potential carriers within families, regardless of the patient's age at the time of testing. Ensuring access to accurate genetic testing for all individuals suspected of having MD, regardless of age, becomes increasingly critical for optimal patient care and advancing research in the field. **Approaches for consideration:** address barriers to genetic testing, including gaps in knowledge and understanding and lack of coverage from public and private payers; explore AI and machine learning to improve diagnostic precision, reduce time to diagnosis, and enable timely intervention, ultimately leading to better patient outcomes.

Universal Access to Specialist Care Across the Lifespan

While many people diagnosed with childhood-onset MDs are living longer, our healthcare system presents significant challenges as those individuals transition to adulthood. Adults living with MDs lose parental insurance coverage at age 26 and multidisciplinary care becomes more complex and specialized as comorbidities arise as the condition progresses. Shortages of specialized clinical experts in adults living with MDs persist. Everyone living with MD, but particularly adults, face challenges in accessing behavioral, psychological, dental, gynecological, urological, and other generally routine care. **Approaches for consideration:** streamline the transition from parental to individual insurance coverage, encourage greater training and geographic distribution of adult MD clinical specialists, and eliminate barriers to telehealth and access to adult multidisciplinary care across state lines, including limited provider networks, coverage and reimbursement denials, and state licensing non-reciprocity.

Mental Health

There is a critical gap in insurance coverage for those living with MD requiring mental health care services. Despite the Affordable Care Act's requirements, many insurance plans impose limitations on mental health services, often not fully covering therapy and other essential treatments (How to Know if Your Insurance Covers Therapy for 2024, Healthline.com; Health Benefits & Coverage, Healthcare.gov; Mental Health Insurance in the US, Anthem.com). Examples of mental health services commonly not covered by insurance include outpatient therapy services, non-hospitalization treatments (day-to-day counseling), and out-of-network providers. The reason that that many mental health professionals do not accept insurance is due to inadequate reimbursement rates. This lack of comprehensive coverage creates significant barriers to accessing necessary mental health care, exacerbating the emotional challenges faced by those living with MD. **Approaches for consideration:** address mental health insurance coverage gaps to ensure holistic care and improve the quality of life for this community.

Addressing Health Disparities

Individuals living with muscular dystrophies vary across socio-economic strata, which can limit their access to 1) costly and timely FDA-approved therapies, especially gene-based and cell therapies, 2) healthcare specialists, 3) resources to increase health literacy, and 4) clinical trials (which leads to non-representative and inconclusive results). Consequently, the socio-economic challenges exacerbate the already significant burden of managing MD, as families often face substantial financial and employment impacts, further hindering their access to necessary care and treatments. **Approaches for consideration:** reduce barriers to research study participation and match the diversity of enrolled participants to that of the population of affected individuals; expand health literacy programs; safety net programs such as Medicaid should ensure access to specialists, innovative treatments and ancillary services; develop programs that cover the costs of travel, lodging, and childcare when visiting specialists should be considered.

Additionally, developing and implementing strategies to ensure equitable access to new treatments once approved is essential. By addressing these generalizability concerns, the MD community can work towards ensuring that advances in treatment benefit all affected individuals, regardless of their socio-economic status or background.

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