

# Acknowledging Voices in Duchenne (AVID) Survey 2024

Exploring the impact of Duchenne muscular dystrophy (DMD)  
and empowering independence



# Maintaining My Freedom: Study highlights the importance of independence for individuals living with DMD<sup>1</sup>

To learn more about the impact of Duchenne Muscular Dystrophy (DMD) on patients ≥12 years old, Sarepta Therapeutics partnered with market research company, The Harris Poll, to conduct an online survey with 158 U.S. participants, including DMD patients aged ≥12 (n=109) and caregivers (n=49). Caregivers were able to take the survey on behalf of the individual they provide care for or help the individual take the survey. The survey was designed to take approximately 15 minutes and was made up of 3 categories: the impact of living with DMD; HCP relationships and communication; and treatment considerations and decisions.<sup>1</sup>

## Background

DMD is a progressive, X-linked neuromuscular disease. It is a rare condition caused by a mutation in the *DMD* gene that results in the absence of functional dystrophin protein in skeletal, cardiac, and respiratory muscle. This leads to progressive muscle weakness, loss of ambulation, respiratory weakness, and cardiomyopathy. The unique challenges that affect older individuals throughout disease progression are often underrepresented.<sup>2-4</sup> By focusing on individuals aged ≥12 years, the AVID survey provides valuable insights for healthcare providers to drive action and support evolving goals in this population.

## Disclosures

The survey was conducted on behalf of Sarepta Therapeutics in collaboration with Elijah Stacy (consultant and adult living with DMD), Team Joseph (a DMD advocacy organization), and The Harris Poll. Team Joseph, Elijah Stacy, and participants were compensated.

## Survey Consultants



**Elijah Stacy**  
*Adult living with DMD*

Elijah Stacy founded Destroy Duchenne, a nonprofit organization, at age 15 to fight for himself and others living with DMD. His organization is dedicated to advancing gene therapies for Duchenne Muscular Dystrophy. As an author and consultant for biotechnology companies, Elijah drives innovations in medical technology, focusing on creating a future where revolutionary treatments improve lives and make history.



**Marissa Penrod**  
*Team Joseph*

Marissa Penrod is a mom of 3 and the founder & CEO of Team Joseph – a nonprofit named for her son, Joseph, who was diagnosed with Duchenne in 2008 at the age of 5. Team Joseph's mission is to help patients and families have the best quality of life possible through direct assistance, and to support research aimed at discovering treatments or a cure for all children and adults living with Duchenne. Marissa is committed to elevating the voices of all families living with Duchenne so they are heard and cared for.

## Key Takeaway 1

# Hear It From Me: Highlighting the importance of maintaining independence and upper limb function (ULF)

Maintaining independence and ULF for as long as possible is a top priority for patients. This is unsurprising, as they report living with DMD has a direct negative impact on their physical and mental health. Ultimately, 91% of patients want to maintain a positive outlook despite living with DMD and 66% feel it is possible to live a full life with DMD. Patients also want others to know that there is much more to them than their DMD.<sup>1</sup>

### Independence shouldn't stop after loss of ambulation

The ability to go about one's day-to-day life and maintain independence matters a lot to DMD patients.<sup>1</sup>

**98%** say it is important to be able to do basic things in their daily life (eg, brush my teeth, go to the bathroom, eat/drink at mealtimes, etc.)

**97%** say it is important to maintain as much independence as possible for as long as possible

**97%** say it is important to maintain as much ULF as possible for as long as possible

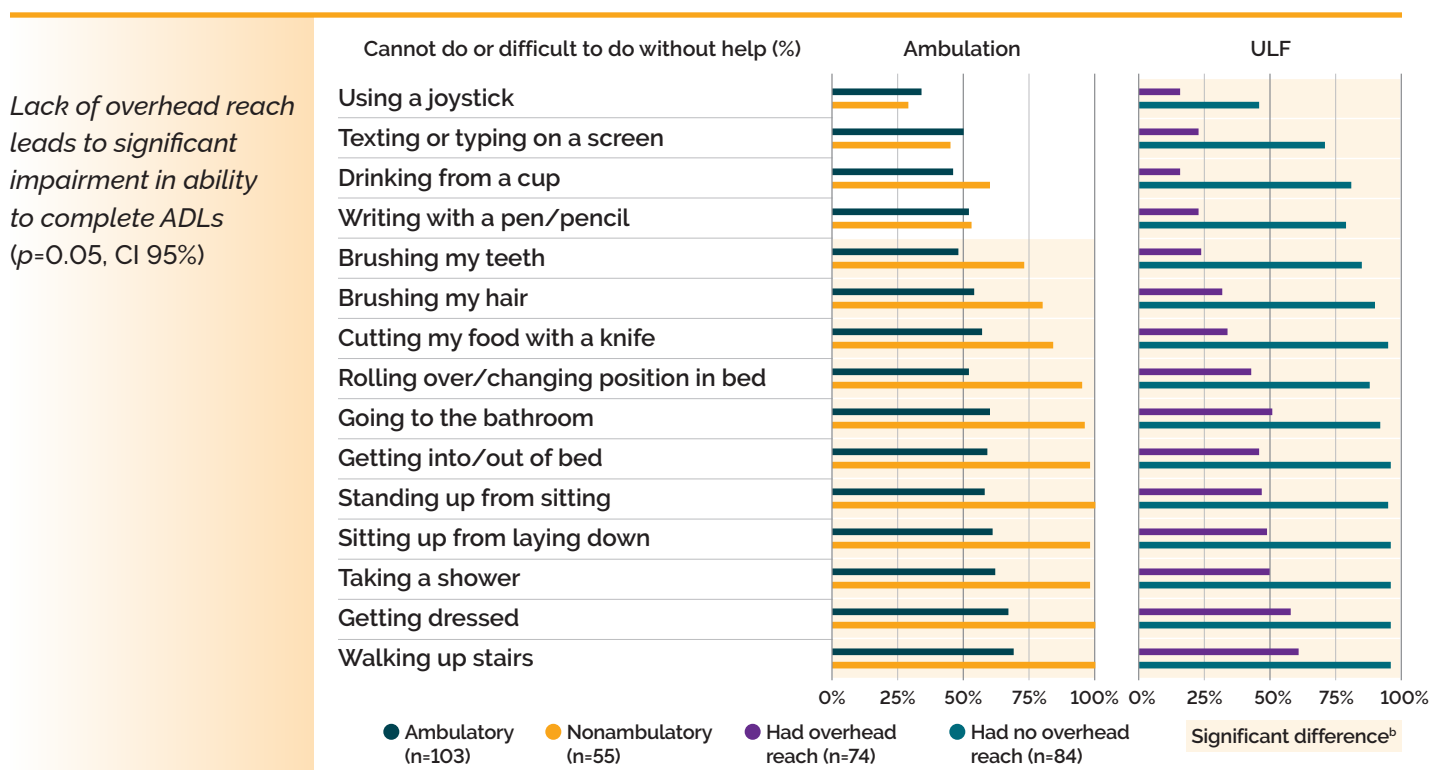
### Patients are impacted by the loss of ULF

Nearly all patients note the importance of retaining ULF for as long as possible. The loss of overhead reach appears to have a considerable impact on their ability to independently complete activities of daily living (ADLs) without help (Figure 1). This impact is felt whether they are ambulatory or not. According to 8 in 10 respondents, the more they lose the ability to physically complete day-to-day activities, the more hopeless they feel. They agree the possibility that they may need help in the future – to do basic things in their life – makes them feel helpless.<sup>1</sup>

"Having ULF is essential to my independence, allowing me to take care of myself and stay connected with others. It enables me to do simple but important things like shaking hands or hugging loved ones, which deeply enhance my quality of life."

—ELIJAH STACY, adult living with DMD

Figure 1. Impact of Ambulation and ULF on Ability to Complete ADLs<sup>1a</sup>



<sup>a</sup>Impact of ambulation and ULF were analyzed separately from the same set of respondents; respondents may be represented in both groups.

<sup>b</sup>Based on the difference reported in ambulation status or reported in ULF status.

## Key Takeaway 2

# Give It to Me Straight: The importance of maintaining open and honest patient communication

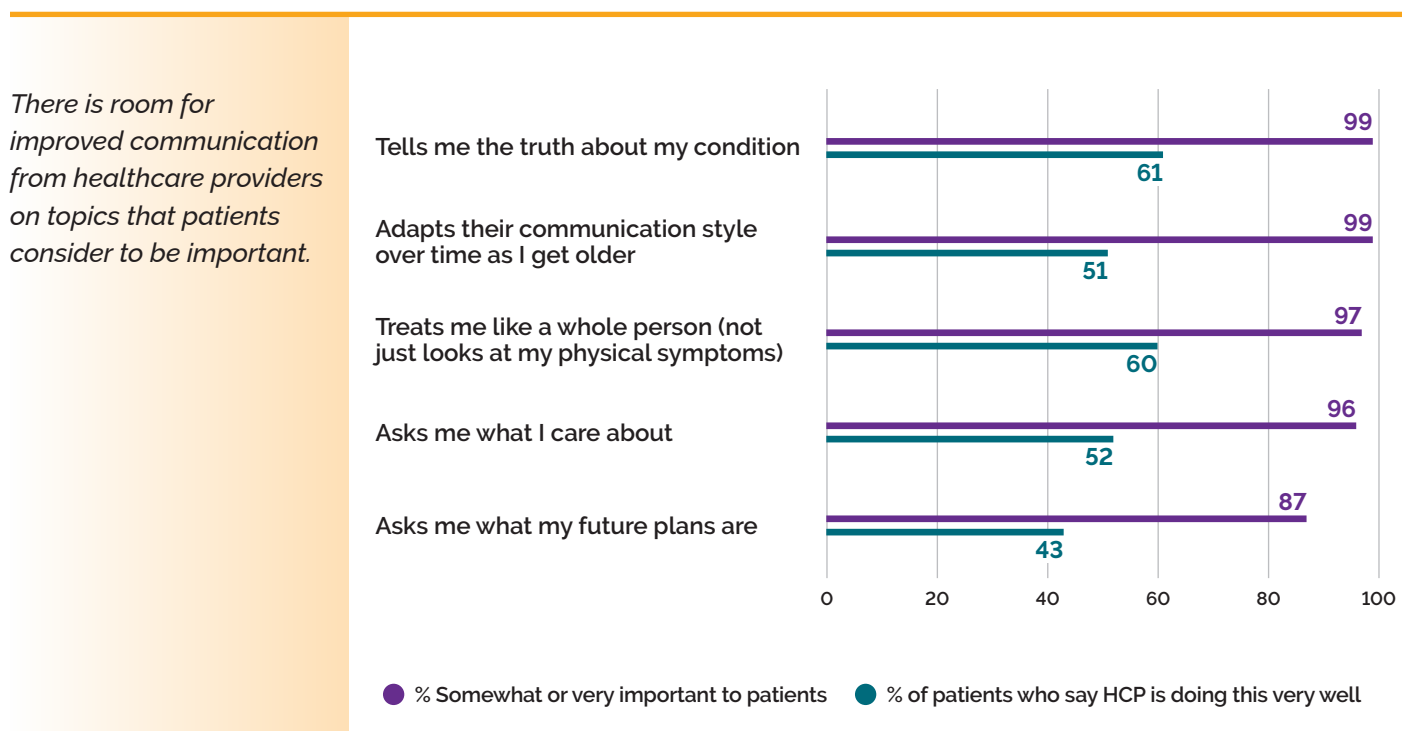
Not only is it important for 96% of DMD patients to feel seen, heard, and respected by their healthcare provider(s) as they get older, most find various aspects related to patient-provider communication to be very important, such as telling them the truth about their condition (67%) and treating them as a whole person (70%).<sup>1</sup>

### Patients highly prioritize the importance of transparent conversations

Healthcare providers telling patients the truth about their condition and treating them like a whole person is important to nearly all and very important to around two-thirds of patients surveyed. While 61% of patients feel that their healthcare providers are effectively communicating the truth about their condition, there is an opportunity to enhance this communication and build stronger patient trust. This can be seen with 29% of patients who feel like their provider just sees their symptoms of DMD and not them as a person.<sup>1</sup>

It is important to patients that HCPs ask what they care about and about their future plans. However, not all patients feel that their HCP does this well, leaving room for improvement. Only 52% of patients believe their HCP is doing very well at asking what they care about, and only 43% believe their HCP is doing very well at asking about future plans. As DMD patients get older, their needs change. Nearly all (99%) of respondents report that it is important that their healthcare provider adapts their communication style over time as they get older, but only 51% report that their provider is currently doing this very well.<sup>1</sup>

Figure 2: Patient Priorities with Healthcare Provider Communication<sup>1</sup>



### Key Takeaway 3

## Tell It to Me Sooner: Proactive conversations help for future planning

Proactive conversations about treatment are highly important to patients. Many wish they had started treatment sooner and are open to knowing more about the treatment options available. This is especially important given the changing priorities and treatment needs based on different life stages and lifestyle changes.<sup>1</sup>

### Knowing more, sooner

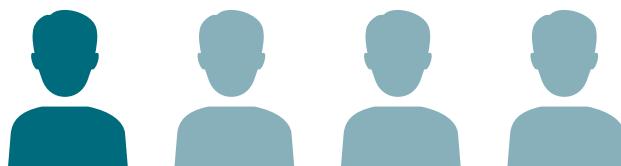
Nearly three-quarters of patients wish they had started treatment for DMD sooner. Although 80% of patients are satisfied with their current treatment, 91% wish they knew more about other treatment options and are open to learning more. 91% of patients also say that they want to know about all available treatments, regardless of whether they are the right fit at the time.<sup>1</sup>

In addition to wanting proactive communication about treatment options, patients wish their HCPs had talked to them sooner about ways to maintain their independence (39%) and ULF (35%).<sup>1</sup>

33% of patients find it hard to discuss what is important to them with their healthcare provider, showing that there is an opportunity to improve how and when these topics are discussed.<sup>1</sup>

### Talking earlier to prepare for the future

While 85% of patients report that they are making plans for their future, only 38% of patients say their HCP is doing very well at asking them what their goals are.<sup>1</sup>



Approximately 1 in 4 patients say that they rarely or never discuss future plans or ways to maintain independence with their DMD provider, which are topics that they would like to discuss or spend more time discussing with their HCP.<sup>1</sup>

For 80% of patients, priorities and needs regarding treatment have changed over time based on different life stages and lifestyle changes. Keeping those discussions dynamic is also important given that treatment needs are ever-changing.<sup>1</sup>

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“There is so much we have to react to as families living with Duchenne. Because this disease is progressive, we are often figuring things out on the fly as our sons' physical condition declines. What may be a good plan of action today, won't be an effective plan tomorrow. I think our quality of life and the joy in our days would be so much greater if our care teams strategized with us on how to plan for the future – on how to address not only what's right in front of us, but what's coming down the road. Every single day, I celebrate that my son has more years ahead than anyone may have thought. But I would love proactive support in figuring out that future before we're scrambling to address his evolving needs.”

—MARISSA PENROD, *Team Joseph*

## Conclusion

### There is an ongoing need for proactive and open communication between healthcare providers and their patients with DMD

Proactive conversations about treatment are highly important to patients. Many wish they had started treatment sooner and are open to knowing more about the treatment options available. This is especially important given changing priorities and treatment needs based on different life stages and lifestyle changes.<sup>1</sup>

#### 99% of patients feel that their HCP telling them the truth about their condition is important.<sup>1</sup>

Despite the importance, there is room for improvement in their care with nuanced conversations that fit to their needs.<sup>1</sup>

#### 97% want to maintain as much independence as possible for as long as possible.<sup>1</sup>

Patients view declining ULF as a core component to the impact of DMD with getting dressed, going to the bathroom, changing positions in bed, and brushing their teeth being areas they see as critical to maintaining independence.<sup>1</sup>

#### 91% of patients wish they knew more about treatment options earlier and want to know about all available options.<sup>1</sup>

Despite the clear challenges of living with the disease, patients believe that it is possible to live a full life with DMD.<sup>1</sup> Healthcare providers and patients who work closely together to have an open dialogue about treatments and priorities can come together to create an action plan with independence in mind.

## Survey methods

A total of 158 participants living in the United States were surveyed, including DMD patients aged ≥12 years (n=109) and caregivers of DMD patients aged ≥12 years (n=49).<sup>1</sup>

### Qualifications

Participant recruitment was conducted by The Harris Poll and Team Joseph. Patient participants were diagnosed with DMD by a healthcare provider, and the diagnosis was self-reported through screener questions. Caregiver participants were involved in a minimum number of care activities. Caregivers were able to take the survey on behalf of the individual they provide care for or help the individual take the survey. Caregivers were instructed to read and answer the questions from the patient's perspective.

### Limitations

Data was not weighted, and therefore only represents the experiences and opinions of those who completed the survey.

### Demographics

Survey demographics reflect the current DMD landscape in the U.S. The mean age of patient participants was 18.8 years, with 21% from age 12-14 (n=33), 52% from age 15-17 (n=82), 4% from age 18-20 (n=7), and 23% at ≥21. Of caregivers, 88% were parents, 6% were other family members, 4% were grandparents, and 2% were friends.<sup>1</sup>

Patients (or caregivers on the patient's behalf) self-reported their functional status based on descriptions provided in the survey. The descriptions used to self-classify ULF were based on the Performance of Upper Limb (PUL 2.0) entry items.<sup>5</sup>

### Statistical significance

Statistically significant differences at the 95% confidence interval for groups of interest (ambulation and ULF) have been included where applicable. Statistically significant comparisons highlight differences in responses between one demographic group of interest vs. their counterpart group – for example, statistically comparing had overhead reach vs. had no overhead reach and (separately) ambulatory vs. non-ambulatory to see if there are unique perspectives between the two groups.



### Functional Status of Patient Participants<sup>1</sup>

	All Respondents (n=158)	% of Respondents
<b>Ambulation Status</b>		
<b>Ambulatory</b>	<b>103</b>	<b>65</b>
<b>Early ambulatory<sup>a</sup></b>	<b>41</b>	<b>26</b>
<b>Late ambulatory<sup>b</sup></b>	<b>62</b>	<b>39</b>
<b>Nonambulatory</b>	<b>55</b>	<b>35</b>
<b>Early nonambulatory<sup>c</sup></b>	<b>38</b>	<b>24</b>
<b>Late nonambulatory<sup>d</sup></b>	<b>17</b>	<b>11</b>
<b>Upper Limb Function</b>		
<b>Had overhead reach<sup>e</sup></b>	<b>74</b>	<b>47</b>
<b>Had no overhead reach<sup>f</sup></b>	<b>84</b>	<b>53</b>

<sup>a</sup>Can walk and can stand up from lying down on the floor independently (i.e., without help).

<sup>b</sup>Can walk but can no longer stand up from lying down on the floor independently (i.e., without help).

<sup>c</sup>Can no longer walk but I do not use assisted ventilation (either at night or during the day).

<sup>d</sup>Can no longer walk and I use assisted ventilation (either at night or during the day).

<sup>e</sup>Can raise both arms at same time above head (either straight or with bent elbows).

<sup>f</sup>No overhead reach was classified as an individual reporting any of the following:

- Can raise both arms at same time, but only to shoulder height
- Can raise plastic cup with liquid to mouth using both hands if necessary
- Can raise one or both hands to mouth but not plastic cup with liquid in it
- Can use hands to hold a pen, pick up a token, or drive a powered wheelchair
- No function of hands at all

**References:** **1.** Data on file. Sarepta Therapeutics, Inc. **2.** Ryder S, Leadley RM, Armstrong N, et al. The burden, epidemiology, costs and treatment for Duchenne muscular dystrophy: an evidence review. *Orphanet J Rare Dis.* 2017;12(1):79. **3.** Cowen L, Mancini M, Martin A, et al. Variability and trends in corticosteroid use by male United States participants with Duchenne muscular dystrophy in the Duchenne Registry. *BMC Neurol.* 2019;19(1):84. **4.** Han S, Xu H, Zheng J, et al. Population-wide Duchenne muscular dystrophy carrier detection by CK and molecular testing. *Biomed Res Int.* 2020; 8396429. **5.** Pane M, Coratti G, Brogna C, et al. Upper limb function in Duchenne muscular dystrophy: 24 month longitudinal data. *PLoS One.* 2018;13(6):e0199223



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