September 30, 2026

Dear Duchenne Community,

Today, Sarepta shared new data at the 2026 World Muscle Society Annual Congress that add to our understanding of ELEVIDYS (delandistrogene moxeparvovec) across different ages and stages of Duchenne. We issued a press release highlighting these presentations.

For families living with Duchenne, every bit of learning matters, especially when new data helps us better understand what treatment may mean for a child not only today, but over time.

One of the analyses presented today looked at 25 boys with Duchenne who received ELEVIDYS between 8 and 12 years of age. This is an important age range because Duchenne can become increasingly difficult for children and families as functional abilities change.

The analysis compared these patients with 99 individuals from well-characterized Duchenne natural history datasets who were carefully matched on important factors that can influence disease progression.

After two years, the ELEVIDYS-treated group had statistically significant differences compared with the external control group across multiple measures of motor function, with the ELEVIDYS-treated group:

- **NSAA:** scoring **3.32 points higher** than the external control group
- **Time to rise from the floor:** having a **0.041 rises-per-second higher rise velocity** than the external control group
- **10-meter walk/run:** having a **0.195 meters-per-second higher velocity** than the external control group

Differences were observed at both one and two years after treatment, providing evidence that the functional benefit seen in this analysis was sustained over the two-year period, similar to previous results in patients who were treated between 4 and 7 years in age.

Importantly, while these particular findings are based on a carefully matched external control analysis, they are aligned with the broader body of evidence supporting ELEVIDYS and its approval, which included placebo-controlled studies. We continue to collect long-term follow-up data to further characterize the effects of treatment over time.

Additionally, we presented data from nine children ages 2 to 3 years who received ELEVIDYS in the ENDEAVOR and ENVOL studies.

At 12 weeks after treatment, these children demonstrated robust ELEVIDYS micro-dystrophin expression and localization in muscle cells. The levels observed were higher, on average, than those previously reported in older ambulatory and non-ambulatory patients.

These findings show that the treatment is reaching muscle cells and producing micro-dystrophin. These children will need to be followed over time to understand the full extent of functional benefit resulting from earlier expression of Elevidys-micro-dystrophin. It is also important to note that the use of ELEVIDYS in children under four years of age remains investigational, and its safety and efficacy in this age group have not been evaluated by regulatory authorities.

Together, these analyses add to the growing body of evidence being generated across different ages and stages of Duchenne. They also reinforce why long-term follow-up remains so important.

For families, treatment decisions are deeply personal and involve weighing the available evidence, potential benefits and risks, individual circumstances, and the guidance of their healthcare team. Our responsibility is to continue generating and sharing meaningful evidence so that families and physicians have the information they need to make informed decisions.

We know that families want more than data points. You want to understand what those data may mean for your child, how they fit with what has been observed in other children, and what we are still learning. We remain committed to listening to the Duchenne community, advancing the science, following patients over time, and sharing evidence transparently as we learn more.

To every individual and family living with Duchenne: thank you for continuing to partner with us in the pursuit of meaningful progress.

Sincerely,

Wendy

Wendy Erler
Senior Vice President, Patient Affairs