



Alex
Living with Duchenne

A new formula for function in Duchenne

Learn how dystrophin quantity, quality, and distribution could add up to **meaningful functional improvements** in Duchenne muscular dystrophy.



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Each boy living with Duchenne and his family facing this should be able to define what autonomy means to them. Autonomy is what's so important to us.

Rachel, mom and caregiver of a 6-year-old living with Duchenne

”

Moving to a more functional future

Living with Duchenne, a rare, complex disease, means working every day to hold on to the abilities that matter most to you. **Important advances have been made to slow progression, but it's time to shift expectations toward meaningful improvements you may see and feel in everyday life:**

more independence, more stamina, more strength.

Progress in Duchenne has only been possible because of the dedication and determination of the community. Dyne is committed to being a trusted partner to families and advocates in continuing this work together.



Throughout this brochure you'll find areas to jot down your thoughts and feelings about living with Duchenne and your hopes for the future.

Share your responses with your doctor and together, make the decisions that could bring the best possible outcomes for you.

Learn about **the impact** that **greater function** could have on the Duchenne community.

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The dystrophin-function connection

The main cause of Duchenne is well-understood: The body does not make working dystrophin, the protein that provides a protective cushion for your muscles during everyday movement.

But simply making more of it isn't enough. Having the right **quantity** (more dystrophin), **quality** (dystrophin close to what the body naturally makes), and **distribution** (effective delivery) of dystrophin—all at the same time—is needed to make meaningful functional improvement possible in Duchenne.

A NEW EQUATION

$$\text{Quantity} + \text{Quality} + \text{Distribution} = \text{Functional Improvement}$$

Q uantity

More dystrophin can mean more protection for the muscles, and may help them stay stronger for longer.

Q uality

High-quality dystrophin contains key parts of the natural protein that the body makes, allowing it to help support the muscles.



D istribution

It needs to reach the most affected places in the body, including skeletal muscle (used for movement and breathing), cardiac (heart) muscle, and smooth muscle (found in the digestive system).

$$\begin{aligned} & \text{Q uantity} \\ & + \\ & \text{Q uality} \\ & + \\ & \text{D istribution} \end{aligned}$$

Functional improvement

Your functional factors

What could functional improvement mean to you?

- Small improvements seen over time
- Delaying loss of ability to walk
- Maintaining function
- Preserving independence

A look at today's dystrophin-producing approaches in Duchenne

In recent years, exon-skipping and gene therapy have focused on addressing the root cause of Duchenne rather than just its symptoms. These advances have helped researchers better understand the **individual components of the equation** that are most important for muscle function.



EXON SKIPPERS

- These medicines showed for the first time that it is possible to produce dystrophin that resembles the natural protein
- Because of the way the medicine is delivered, it may not reach all the muscles affected by Duchenne that need it most

GENE THERAPY

- Gene therapy is designed to deliver “instructions” directly to muscle cells so they can make dystrophin
- It produces a large amount of a shorter version of dystrophin called micro-dystrophin
- Gene therapy is given once and may not be re-dosed. As the body naturally grows, new muscle cells impacted by Duchenne may not receive the medicine

While progress has been made, achieving all 3 components of dystrophin—quantity, quality, and distribution—at once is still a challenge in Duchenne treatment. Treatments that only address some components of dystrophin may not be enough for some patients to lead to improvements in function.

Your functional factors

If dystrophin could work better throughout the body, what changes in daily life would you find most impactful?

How it could add up for people living with Duchenne

Research is increasingly focused on **achieving all 3 components—quantity, quality, and distribution**—with the goal of supporting the functional improvements that matter most to people living with Duchenne and their families.

People living with Duchenne and their families look toward shared goals that reflect meaningful improvement.



PRESERVING DAILY FUNCTION

- Maintaining the ability to walk
- Improving use of arms and hands
- Supporting self-care and independence at school, at work, and in social settings



PROTECTING VITAL ORGANS

- Preventing or delaying heart and muscle weakness
- Preserving breathing capacity



CHANGING THE COURSE OF DUCHENNE

- Delaying progression to more advanced stages of the disease, including:
 - Loss of movement
 - Breathing complications
 - Worsening heart and breathing function



Upper body function would help me a lot in my day-to-day with reaching for things and lifting things.

Jake, 23-year-old living with Duchenne



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Take a moment to assess today's realities and tomorrow's possibilities.

Write your responses in the designated areas below.

If you're living with Duchenne:

If you care for someone living with Duchenne:

Today, I'm feeling a bit challenged by:	New approaches in scientific advancement in Duchenne make me feel:
I feel optimistic about new approaches because:	If dystrophin quantity, quality, and distribution could be achieved together, I would most want to see gains in:
If I was able to achieve functional improvement, what I would want to try most is:	With a little more functional ability for them, I could:
My greatest wish for the Duchenne community is:	I stay informed about scientific research and advancements in Duchenne by:



Bring these notes to your next doctor's visit to help start a conversation about your goals and what meaningful function looks like for you.



Kyrie
Living with Duchenne

Shared decisions for a **focused goal**

Together, you and your doctor will make decisions that are most appropriate for you.

Your functional factors

- **Discuss with your doctor** what improvements would make the most difference in your everyday life
- Have a conversation about the 3 key components of dystrophin: **quantity, quality, and distribution**
- **Talk about recent advancements in Duchenne** to stay up to date with the latest information

Thank you to the Duchenne community.

Your partnerships and insights help guide scientific advancements for people living with Duchenne muscular dystrophy.



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