For people with

DUCHENNE MUSCULAR DYSTROPHY

increasing dystrophin is vital

We Speak DUCHENNE™

This is Emmett, and he is living with Duchenne.
Emmett is a paid ambassador.

Dystrophin plays an important role in muscle function

In order to function properly, our muscles need a vital protein called **dystrophin**.

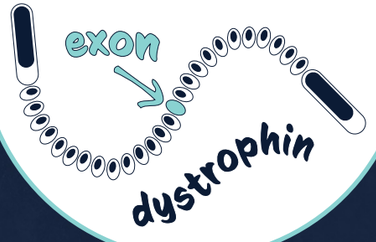
Dystrophin is a key part of a group of proteins that all work together to strengthen and protect muscles as they contract and relax.

Duchenne muscular dystrophy (also known as "Duchenne" or "DMD") results from muscles not having enough dystrophin.

Duchenne is caused by a genetic mutation in the dystrophin gene ("DMD gene"). This gene has 79 individual sections, called **exons**.

An **exon** is a region of DNA with genetic information for making a protein.

Each **exon** makes one piece of our body's **dystrophin protein.**



People with Duchenne lack dystrophin

Due to a genetic mutation, people with Duchenne are missing one or more exons from their DMD gene. ***This prevents their body from making usable dystrophin.***

Without usable dystrophin,
people with Duchenne gradually lose healthy muscle tissue.
This leads to common symptoms:

Muscle weakness

Balance issues

Serious health problems over time

Various treatments
are used to help manage the symptoms of Duchenne:

Steroids for inflammation

Physical therapy for orthopedic issues

Medical management for heart or lung complications

However, these treatments don't increase dystrophin.

Increasing dystrophin may slow the progression of Duchenne

Since a person with Duchenne has low levels of dystrophin (or none at all), increasing their dystrophin may slow the progression of their DMD. This has been shown in clinical studies.

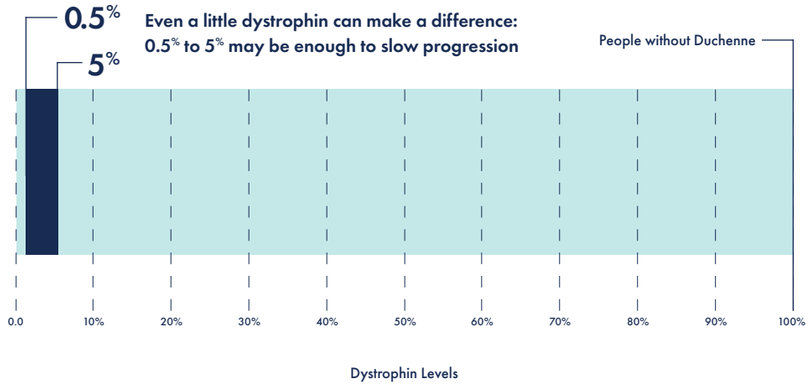
When treating Duchenne, time can be critical

Ideally, **increasing dystrophin in a person with Duchenne should happen as soon as possible**, since the more time passes, the more muscle is lost.

When developing a treatment plan for a person with Duchenne, knowing their genetic mutation is vital. ***Ask their doctor about their specific genetic mutation as soon as possible.***

A little dystrophin goes a long way

*Research suggests that having dystrophin levels of **just 0.5% to 5% of normal** may be enough to **slow the progression of Duchenne** in some people.*



When treating Duchenne, increasing dystrophin as soon as possible after diagnosis can be a positive step forward

This is Brantley, and he is living with Duchenne. Brantley is a paid ambassador.

How can dystrophin be increased in people with Duchenne?

Currently, two different types of therapy can be used to increase dystrophin in people with Duchenne:

EXON-SKIPPING THERAPY

Helps a person with Duchenne bypass their genetic mutation to create a usable dystrophin protein within their own body.

With exon-skipping therapy, the muscle cells of a person with Duchenne make a blueprint for their dystrophin protein that's **84-97%* as long as the full-length dystrophin** made by someone without DMD.

EXON-SKIPPING DYSTROPHIN
84-97%*

Each type of exon-skipping therapy works for a specific genetic mutation(s). A simple genetic test can help determine if there is an approved exon-skipping therapy for a person with Duchenne.

*For the most common mutations

GENE THERAPY

Inserts an engineered micro-dystrophin gene into a person with Duchenne to help their muscle cells produce a micro-dystrophin protein.

With gene therapy, the muscle cells of a person with Duchenne make a blueprint for their micro-dystrophin protein that's **32-40% as long as the full-length dystrophin** made by someone without DMD.

ENGINEERED MICRO-DYSTROPHIN
32-40%

Eligibility for gene therapy is determined by a patient's doctor. Gene therapy is still being studied for its long-term safety and efficacy.

Duchenne is complicated.
But understanding it doesn't have to be.

We Speak Duchenne is an educational platform that helps break down information about DMD.

Scan the code for helpful information about Duchenne

Or visit **WeSpeakDuchenne.com**

We Speak DUCHENNE™

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