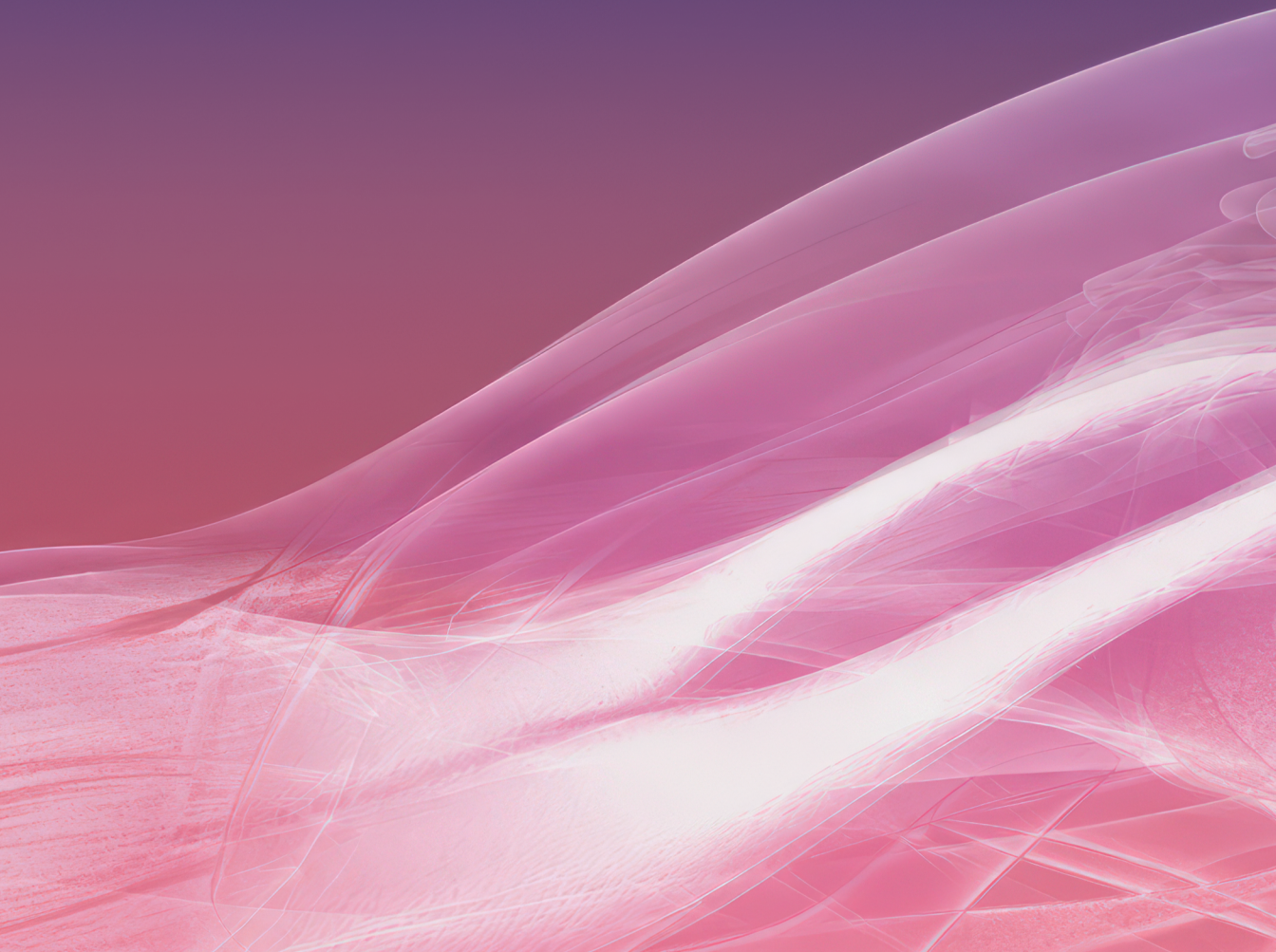


To address the many effects on the body,

Managing Duchenne muscular dystrophy may involve multiple treatments working together



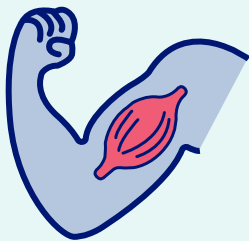
Looking deeper at Duchenne

Understanding what drives muscle damage can help shape care.

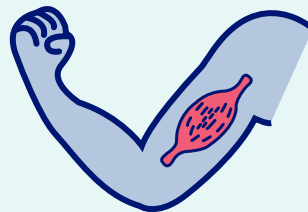
It's widely known that Duchenne muscular dystrophy is a rare, progressive genetic condition that affects muscles used for movement, breathing, and heart function.

But, to understand what's really going on, it helps to look deeper into the muscle, starting with a protein called **dystrophin**. In Duchenne, a genetic variant **prevents the body from making dystrophin, which means it's harder for muscles to stay healthy**. When dystrophin is missing, it causes changes in muscle cells that affect how muscles work.

HEALTHY MUSCLE
with dystrophin



MUSCLE IN DUCHENNE
without dystrophin

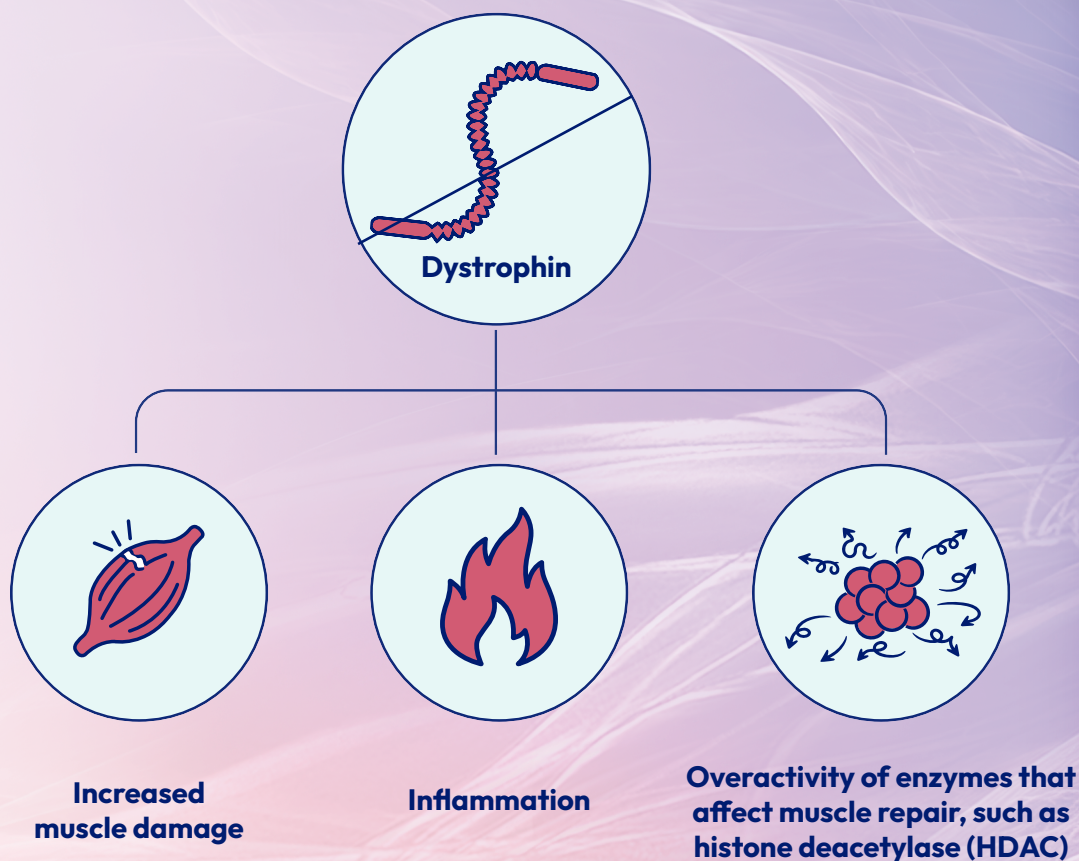


Because these changes can affect the body in different ways, care may include more than one type of treatment, each helping to address a different effect of the disease.

The effects of missing dystrophin

Without dystrophin, muscle fibers are more fragile.

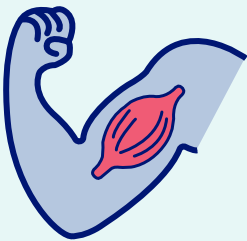
Absence of dystrophin in the muscle leads to:



Dystrophin loss sets off a series of changes that affect the ability of muscles to repair themselves, leading to muscle loss over time.

Understanding HDAC in healthy muscle

HDAC is an enzyme in the body's cells that **helps control how muscles function and repair themselves.**



Healthy muscles experience small tears every day.



When muscles are damaged, the body sends signals that help repair and rebuild muscles so they can grow stronger.



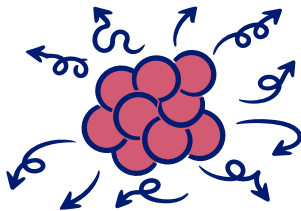
HDAC helps keep these repair signals balanced, allowing muscles to heal, grow, and work properly.



HDAC plays an important role beyond dystrophin in helping muscles stay strong and healthy.

Understanding HDAC in Duchenne

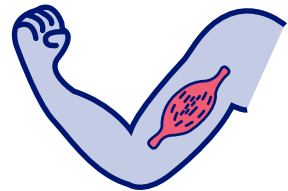
Although HDAC plays an important role in healthy muscle, **the absence of dystrophin in Duchenne causes HDAC to become overactive**. This disrupts the balanced repair signals that muscles need to heal properly.



When HDAC becomes overactive, it disrupts the signals needed to repair everyday muscle damage.



Over time, damage builds up along with inflammation and scar tissue.



As this damage increases, muscles become weaker over time.



Overactive HDAC disrupts the body's ability to repair muscle, leading to a buildup of damage.

No single treatment addresses every aspect of Duchenne

There are several approved therapies available to help manage Duchenne. In some cases, healthcare providers may use more than one therapy to help support muscle function over time. Supportive treatments may also help manage heart and lung health, mobility, and other symptoms.

Your healthcare team can help determine which treatment approach may be right for you.

Since Duchenne causes multiple changes in muscle cells that contribute to muscle damage, therapies are designed to target these changes in various ways.

The next page shows how these treatments address the changes caused by Duchenne.



Talk with your care team to better understand how multiple treatments working together can address the many effects of Duchenne and help preserve function.



STEROIDS

Steroids aim to reduce inflammation in the muscles.

By calming this inflammation, they can help protect muscles and slow muscle weakness.

They are often used as a starting point in Duchenne care.



EXON SKIPPERS

Exon-skipping therapies help the body make a shorter form of dystrophin by working around certain genetic changes.

This treatment can only be used in patients with specific genetic variants. While they don't fully replace dystrophin, they may help muscles function better.



GENE THERAPY

Gene therapy is designed to help the body make a shorter version of dystrophin.

Not everyone is able to receive gene therapy, and doctors will run certain tests, like checking for antibodies in the blood, to see if it is an option. While gene therapy does not fully replace dystrophin, it may help support and stabilize muscle function.



HDAC INHIBITOR

An HDAC inhibitor is designed to reduce HDAC overactivity and can be used regardless of genetic variant.

In reducing HDAC overactivity, it may also help reduce inflammation and buildup of fat and scar tissue, and support the body's ability to repair and maintain muscle.

Every person with Duchenne is unique. Treatment can be, too

Talk to your care team about different treatment options and how multiple treatments may be used together as part of your Duchenne care plan. Here are some questions you may want to ask:

☐

Should I consider using more than one type of Duchenne treatment?

☐

How could my genetic variant impact which Duchenne treatments may be options for me?

☐

Beyond treatments that target dystrophin, what other approaches may be appropriate for me?

☐

What other important factors should I consider when deciding which treatments are right for me?

☐

What should I know about the benefits and risks of the treatment options outlined in this brochure?

☐

How may insurance coverage, approvals, or out-of-pocket costs affect my treatment options?

Notes from my care team:

Treatment options discussed:

Next steps:

Date of next appointment:

Additional questions to ask my doctor:



Scan to learn more about Duchenne
and different options for care.

Created by ITF Therapeutics in collaboration with these
Patient Advocacy Groups to help support those living with Duchenne:



Parent
Project
Muscular
Dystrophy