Managing Duchenne

10 important THINGS TO KNOW

following a DUCHENNE muscular dystrophy diagnosis

ETHAN, lives *with* Duchenne.
Lives *for* sunny days with his park buddy.

SAREPTA THERAPEUTICS

If your child has recently received a Duchenne diagnosis, you probably have a lot of questions and may be feeling overwhelmed.

Remember that you are not alone. There are many resources available to help you learn more about Duchenne, as well as a large community of families living with Duchenne that can provide support. While seeking education about Duchenne, it can be helpful to start with what's important to know right now. The information that follows can help you begin to understand your child's new diagnosis.

FINN, living with Duchenne

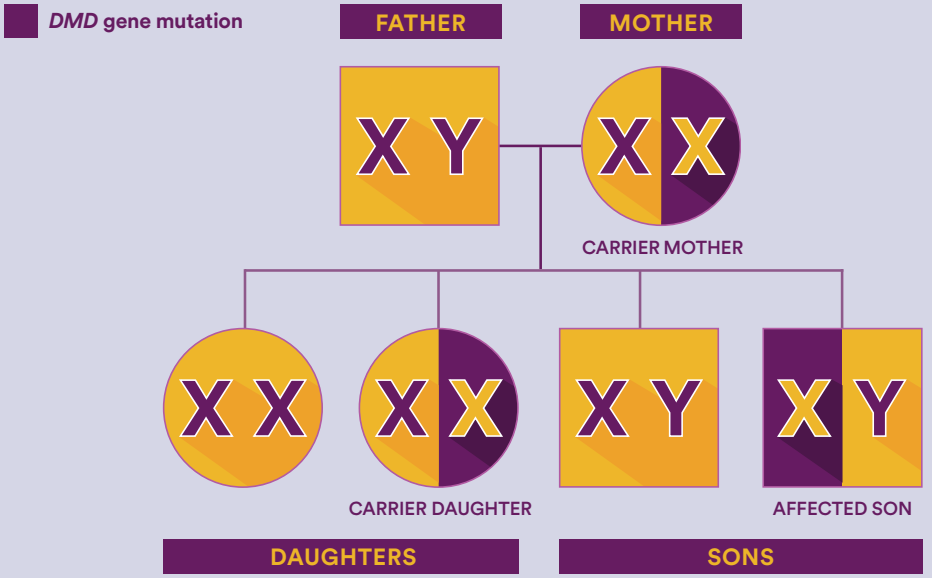
UNDERSTANDING DUCHENNE

1 What is Duchenne?

Duchenne muscular dystrophy, or simply Duchenne, is the most common muscular dystrophy in children. Duchenne affects mostly males, about 1 in every 3500-5000 newborn boys, and impacts families across all races and cultures. Duchenne is a rare genetic condition caused by a mutation in the *DMD* gene, which stops it from producing dystrophin, a protein that helps keep muscles healthy. Dystrophin acts like a shock absorber when muscles contract. Without dystrophin, the muscles become damaged and weaken over time, and may not be able to repair themselves from injury.

A **genetic condition** is caused by a mutation or mutations in a gene. In Duchenne, the mutation is usually inherited, though it develops spontaneously in about 1 out of 3 cases.

How Duchenne is inherited:



Two-thirds of people living with Duchenne inherit the mutated *DMD* gene from their mother. In the other third, the mutation occurs spontaneously.

2 How does Duchenne affect the body and how will that change over time?

Muscles become damaged and inflamed

The *DMD* gene is the largest known human gene and is prone to developing mutations. A mutation in the *DMD* gene can affect the production of dystrophin, which is essential for muscle function. Without proper dystrophin production, the muscles may become inflamed, weak, and damaged, or unable to function as intended.

Other important organs in the body can also be affected

Cardiomyopathy, an enlarging, thickening, or stiffening of the heart muscle that can make it difficult for the heart to pump blood

Continuous, progressive decline in lung function as early as age 5

Psychosocial problems, including difficulties with thinking, learning, and behavior, as well as speech delays

Nutritional problems due to difficulty swallowing, acid reflux, persistent constipation, or buildup of gas in the organs that help digest food

Duchenne progresses over time

Early muscular signs and symptoms

Inability to walk by 16-18 months, difficulty speaking clearly, frequent falls, trouble with running and climbing stairs, muscle pain, cramping, enlarged calves, walking on toes, or using hands to "walk up the body" when trying to stand (referred to as ***Gower's maneuver***)

Loss of the ability to walk

Muscles of the lower limbs closest to the body are affected first, followed by the shoulder muscles, and muscles closest to the end of the limbs

Wheelchair use

Prolonged sitting in a wheelchair may result in increased risk of arm and leg fracture, motion-limiting joint stiffness, and a sideways curve of the spine known as scoliosis

What are the current options to help manage Duchenne?

While currently there is no cure for Duchenne, there are options that may help delay disease progression. Steroids are the most commonly used management option to slow down the effects of Duchenne. They work by reducing swelling and inflammation in the muscles, helping the heart and lungs stay strong, and may be able to help children stay mobile longer. While steroids are widely used and can have a number of positive effects on people living with Duchenne, it's important to discuss additional options.

Here are some terms you may hear your doctor mention when discussing how to manage your child's condition:

RNA Therapy

Also called exon-skipping therapy, this is a group of treatment options available for patients with certain genetic mutations of Duchenne. This type of treatment allows the body to skip over missing exons in order to fit the remaining exons together.

Clinical Trials

Clinical trials study the safety and effectiveness of investigational drugs that are not yet approved by the Food and Drug Administration (FDA).

Ask your doctor what options are appropriate for your child.

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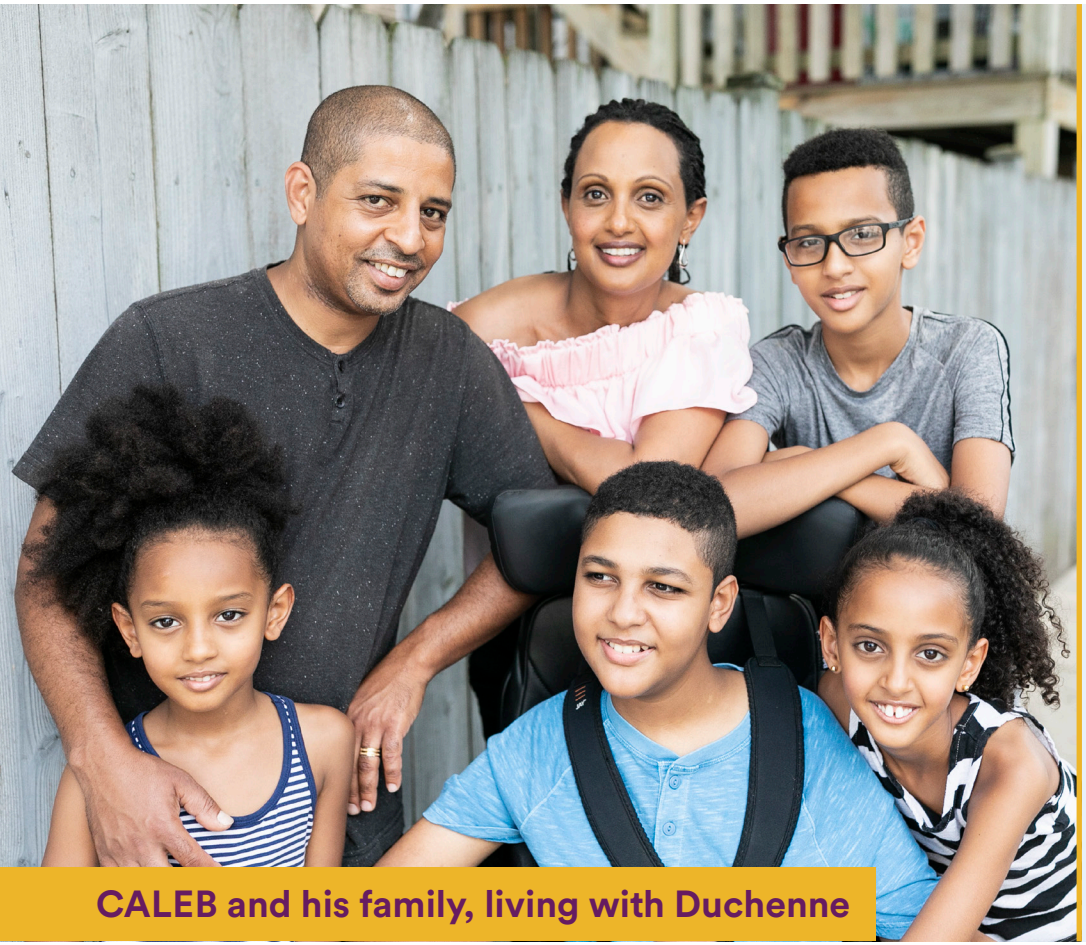
What will the future look like for my child living with Duchenne?

While the average child is diagnosed at about 4 years old, the age of diagnosis and symptoms may widely range. While Duchenne affects each child in a different way, understanding how the disease progresses and how it may impact your family over time can help prepare you and your family for the future.



CALEB and his family, living with Duchenne

The impact of Duchenne on families

Over time, people living with Duchenne become more reliant on help for many everyday activities, including dressing and eating. Individuals living with Duchenne may experience isolation, insecurity, hypochondria, depression, and anxiety.

Duchenne can also affect quality of life for others in the family. For example, the parents of children with Duchenne may experience periods of grief. These feelings can occur when a child reaches a disease milestone like loss of mobility.

Each family member may react differently to the challenges of Duchenne, so it is important to be supportive and remember that it takes time to adjust to these changes. Check in with each other, and don't be afraid to lean on friends and family for support.

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How can my family and I stay organized and manage day-to-day life with Duchenne?

For families caring for a child with Duchenne, life can get busy. Ensure you have the right support by reaching out to patient advocacy organizations and community groups. You can find an extensive list of helpful resources at **Duchenne.com/resources**

Manage and organize medical information and share it with your family. Find tools and apps to help you stay organized through various advocacy groups.

Children's books on DMD
Help your child, your friends, and your family better understand Duchenne.

Find resources to help you work with your child's school to develop an Individualized Education Plan (IEP).

Foster your child's social life by coordinating play dates and time at camp. There are more than 80 short programs for children with muscular dystrophies sponsored by various organizations, including the **Muscular Dystrophy Association** (MDA), **Jett**, and **OneRare**.

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How can I make our home more accessible for my child?

Each person living with Duchenne has different needs. Many children use assistive devices like night splints, scooters, or wheelchairs to get around. When the time is right and your child starts to become more dependent on these devices, it might help to make some home modifications, including widening doorways and installing ramps or stair lifts. Grab bars are also helpful for getting in and out of the shower, bed, or wheelchair.

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Is financial assistance available to help modify my home?

While it's important to remodel your home to better accommodate your child with Duchenne, it can also be expensive.

FINN, living with Duchenne, and his brother

To help pay for these modifications, you can apply for grants.

Advocacy groups can connect you with organizations to help you get the funding you need. View more resources and learn about patient advocacy organizations that support families living with Duchenne at **Duchenne.com/resources**

WE REMODELED OUR HOUSE TO MAKE IT HANDICAP ACCESSIBLE... IT'S A LEARNING CURVE...WE'RE CONSTANTLY LEARNING WHAT TO EXPECT NEXT WITH THIS CONDITION.

—DAWN, WHOSE SON IS LIVING WITH DUCHENNE

ETHAN, living with Duchenne

8 How can I help my child's school provide the best learning experience?

Education is important, and schools may need help adjusting to ensure your child feels safe and comfortable in the classroom. Engage with teachers and administrators and provide them with information about Duchenne, so they can better prepare their classrooms and educate your child's classmates. If your child presents with early developmental delays, you may also consider looking into Early Intervention Programs or Individualized Education Plans. These programs offer children with developmental delays support and services for their individual needs.

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Where else can I find support?

Your child's care team will be there to partner with you and your family every step of the way. A multidisciplinary Duchenne care team may include numerous people, each specializing in different areas. Your child's care team will be structured to your family's needs and is typically led by a neuromuscular specialist.

DILLON, living with Duchenne

A Duchenne care team may include the specialists below:

CORE TEAM

Neurologist/Neuromuscular Specialist
Pediatrician
Care Coordinator

ADDITIONAL SPECIALISTS

Cardiologist
Pulmonologist
Endocrinologist
Psychiatrist

EXTENDED CARE TEAM

Occupational Therapist
Neuropsychologist/Psychologist
Speech/Language Therapist
Physiatrist/Physical Therapist
Genetic Counselor

Support doesn't stop with your care team

The Duchenne community is compassionate and inspiring. Numerous advocacy groups, online communities, and other families can help provide a voice for those living with Duchenne.

CALEB, living with Duchenne

Visit **Duchenne.com** to find ways to get involved and become part of the supportive Duchenne community.

You can also learn more at:

CureDuchenne
https://www.cureduchenne.org ▸

Jett Foundation
https://www.jettfoundation.org ▸

Little Hercules Foundation
https://littleherculesfoundation.org ▸

Muscular Dystrophy Association
https://www.mda.org ▸

Parent Project Muscular Dystrophy
https://www.parentprojectmd.org ▸

Team Joesph
https://www.teamjoseph.org ▸

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When discussing Duchenne, my doctor has mentioned Sarepta. How can Sarepta support my family?

Sarepta has been committed to helping people with muscular dystrophy for decades, offering treatment options for patients with certain genetic mutation types of Duchenne, patient support, and resources. One of those resources is **Duchenne.com**, a website created for the Duchenne community to be a source of knowledge, connection, and hope.

SAREPTASSIST

SareptAssist (**sarepta.com/sareptassist**) teams up a patient and their family with a specialized Case Manager to help coordinate care.

Your SareptAssist Case Manager can help you:

- Better understand how insurance benefits work
- Find treatment locations
- Acquire long-term support throughout treatment
- Receive information about financial assistance programs
- Manage in-office or at-home treatment

For questions about SareptAssist, call 1-888-SAREPTA (888-727-3782). Case Managers are available Monday–Friday from 8:30 AM–6:30 PM ET.

Managing a condition like Duchenne can take some time. However, with the right information, care team, and support, you can learn how to care for your child while also becoming a part of the incredible Duchenne community.

Speak to a doctor to find out what treatment options may be right for your child.

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