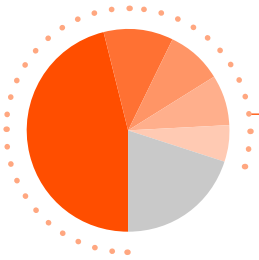
In Duchenne muscular dystrophy (DMD)

Know your or your child's gene mutation and what it may mean for treatment



Up to **80%** of people with DMD may be amenable to current or future exon-skipping approaches

Percentage of *DMD* gene mutations amenable to exon skipping

- ~46% other exons
- 11% Exon 51
- 9% Exon 45
- 8% Exon 53
- 6% Exon 44
- ~20% not amenable to skipping

Amenable means a specific gene mutation may make it possible for a person to be a match for certain treatments

ACT on what you know

Remembering "ACT" can help guide conversations with your DMD care team and support informed treatment discussions.

A — Ask about gene mutation

Do you have access to the genetic testing results?

Genetic test results may already be in the medical record. Genetic testing is usually part of a DMD diagnosis and looks at the specific dystrophin gene ("*DMD* gene") mutation. **Ask your DMD care team to find the results and review them with you.**

Knowing the mutation can help you and the DMD care team better understand the condition. **As DMD treatment options evolve over time, it's important to remember and revisit these results.**

Know the gene mutation?

Use this helpful tool to see your or your child's amenability status.

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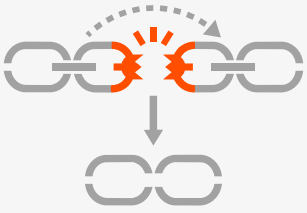
C — Check amenability status

Is the gene mutation amenable to exon skipping?

If you know the *DMD* gene mutation, you can see whether it may be amenable to exon skipping.

Exons are sections of the *DMD* gene that provide instructions for making dystrophin, a protein that acts like a shock absorber to protect muscles from damage during movement

Exon-skipping medications skip certain exons so the remaining exons can fit together, which may allow the body to make working dystrophin



T — Talk with your DMD care team about what these results mean

What do these results mean for my or my child's care?

Continuing the conversation with your DMD care team is important.

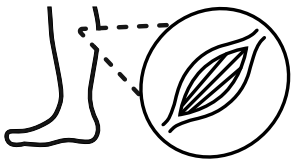
Questions you can ask to start a productive conversation include:

- Do you know my or my child's genetic mutation?
- Can I have a copy of the genetic testing report?
- Are you using amenability status to determine the right treatment option?
- What treatment options are there for this gene mutation?
- Could an exon-skipping medication be an option today or in the future?

In Duchenne muscular dystrophy (DMD)

Overall muscle health matters

Knowing the gene mutation can help inform treatment conversations. Understanding dystrophin and creatine kinase (CK) may provide additional insight into muscle health.

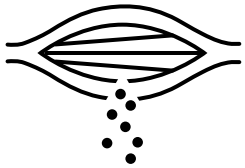


What is dystrophin and why does it matter?

Dystrophin is a protein that acts like a shock absorber to protect muscles from damage during movement. In DMD, the body cannot make enough of its own dystrophin. That's because there is an error, or mutation, in the *DMD* gene.

Without enough dystrophin, **muscle cells become fragile and can be damaged** more easily during everyday movement.

Helping the body make more dystrophin is an important part of treating DMD.



What is CK and what does it show?

CK is a substance found inside muscle cells. When muscle cells are damaged, CK leaks into the blood.

High CK levels can be a sign of damage to the muscles. CK is often one of the first things doctors check if they think a child may have DMD.

What do dystrophin and CK tell us about muscle health?

Dystrophin helps keep muscles strong and protected. CK can be a sign that muscle damage is happening. That's why helping the body make more dystrophin is an important treatment goal in DMD.

Have questions? We're here to help.

Connect with a Novartis Patient & Community Liaison for support.

Call: 1-888-DMD-PCL7 (1-888-363-7257)

Available Monday through Friday, 9 AM to 5 PM ET

Explore UnderstandingDMD.com to learn more about DMD.

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