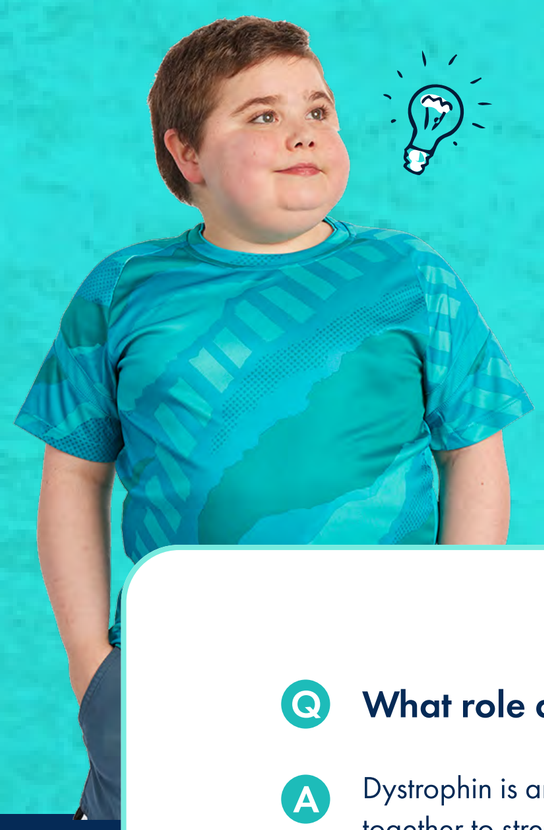

We Speak DUCHENNE™

COMMON QUESTIONS AND ANSWERS

Q **What role does dystrophin play in Duchenne muscular dystrophy?**

A Dystrophin is an essential protein for muscle function. It's part of a group of proteins (a "protein complex") that all work together to strengthen and protect muscles as they contract and relax. People with Duchenne have a genetic mutation that prevents their body from making usable dystrophin; and without usable dystrophin, their muscles are more sensitive to damage and injury and thus get progressively weaker, leading to mobility issues. Since the heart is a muscle and muscles also help support the lungs, Duchenne can lead to serious health issues over time.

Q **Are there ways to increase dystrophin in people with Duchenne?**

A For a person with Duchenne, dystrophin can be increased using two different types of therapy: exon-skipping therapy and gene therapy. Each therapy allows the person's body to create a shorter but functioning dystrophin protein. Exon-skipping therapy makes a dystrophin protein that's 84% to 97% (for the most common theoretical mutations) as long as the dystrophin protein made by someone without DMD. Gene therapy makes a micro-dystrophin protein that's 32% to 40% as long as the dystrophin protein made by someone without DMD.

Q **What is exon-skipping therapy?**

A Exon-skipping therapy is one way to increase dystrophin in a person with Duchenne. By "skipping" a section of the dystrophin gene, it helps the person make usable dystrophin within their own body. Exon-skipping therapy is given to people on a regular basis (such as every week) through intravenous (IV) infusion, which can sometimes be given in the person's home.

Q **How do I know if my child with Duchenne can get (is amenable to) exon-skipping therapy?**

A The dystrophin gene has 79 individual sections, called exons. Each exon-skipping therapy works by "skipping" a specific exon, which helps the body correct for certain mutated sections of exons. These mutated sections are identified by a range of numbers (or a single number). A simple genetic test can help identify the mutated sections of exons. Speak with a doctor to learn if your child is amenable to exon-skipping therapy.

Q **What are the differences between exon-skipping therapy and gene therapy?**

A Each therapy works in a different way to help a person with Duchenne. Exon-skipping therapy helps the person's body bypass their existing genetic mutation, while gene therapy inserts a new, engineered micro-dystrophin gene into the person. Another difference between these therapies is the actual length of the dystrophin protein that is made. With exon-skipping therapy, the dystrophin protein made is about 84-97% as long as the full-length dystrophin (for the most common mutations) protein made by someone without DMD. With gene therapy, the dystrophin protein made is about 32-40% as long as the full-length dystrophin protein made by someone without DMD.

Q **How can I find out about currently available therapies for Duchenne?**

A There are many things to consider when evaluating treatment options. Some therapies can potentially work for specific mutations. Being eligible for (known as being "amenable to") an exon-skipping therapy for Duchenne depends on which specific mutation is causing your loved one's condition. A simple genetic test may help determine if there is an approved treatment for a person with Duchenne. The best way to find out about currently available therapies for Duchenne is to ask a doctor. You can also do an online search for exon-skipping therapies, if you know your child's specific gene mutations.

Q **When should we start therapy to increase our child's dystrophin?**

A Increasing dystrophin in a person with Duchenne may slow the progression of their DMD so it is always better to initiate treatment at an early age, as therapies that increase dystrophin in a person with Duchenne may provide the greatest effect before muscles become too week. Research suggests that having dystrophin levels that are even 0.5% to 5% of normal (100%) may be enough to slow the progression of Duchenne in some people. And dystrophin-increasing therapy can be started at any age, in appropriate patients, even years after the initial diagnosis.

Q **How can I talk to my child's doctor about treatment options for their Duchenne?**

A First, you can ask their doctor if your child's specific genetic mutation(s) may make them amenable to an exon-skipping therapy that can increase their dystrophin. You can also ask if gene therapy may be an option.

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