Considering gene therapy?

Start by talking to your doctor

Gene therapy is a way to treat genetic conditions, which are caused by changes (called *mutations*) in the body's genes. It has been researched for decades, and treatments have been approved for a variety of diseases.

It's also being studied in Duchenne. Even when treating the same disease, each gene therapy is different. That's why it's important to understand how each one works and who may be eligible. This discussion guide can help you prepare for a conversation with your doctor and can even be printed and brought to your appointment.

What is gene therapy?

What is the goal of gene therapy? How does it work?

If gene therapy works as it's supposed to, how might that impact my child's life?

How is it given?

How long does the treatment process take? What is the overall time impact on my family?

What have we learned from clinical trials?

Gene therapy is being studied extensively in Duchenne. What has been learned so far?

Who was included in these trials? Was there anyone like my child?

Are there any safety concerns? What are the risks of having gene therapy?

What kind of monitoring is required after receiving gene therapy?

Who is gene therapy appropriate for?

What tests are needed to determine if my child is eligible?

What do antibodies have to do with eligibility?

Is gene therapy intended for specific mutations?

If my child receives gene therapy, what could that mean for our future treatment options and clinical trial opportunities?

What are my options if my child is not eligible?

Additional questions I have:

Looking for more information on gene therapy? Visit Duchenne.com/genetherapy.

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