

Clinical Trial Resources on the DuchenneConnect Registry

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Available Resources:

1. Trial Finder Tool
2. Clinical Trial Decision Guide
3. Clinical Trial Video Library
4. Duchenne Deletion Tool

Parent Project Muscular Dystrophy

JOIN THE FIGHT. END DUCHENNE.



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[Sarepta E88ENCE Trial Recruiting](#)

[Nationwide Carrier Study](#)

[BM3-888088 Global Clinical Trial Recruiting Soon](#)

FUND RESEARCH

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Clinical Trials

Read our family-friendly FAQs on clinical trials

[Click here to register now!](#)



Decode Duchenne

Free genetic testing, interpretation and counseling for individuals with Duchenne or Becker muscular dystrophy. Decode Duchenne is administered by DuchenneConnect and PFMD and is funded by Sarepta Therapeutics and PTC Therapeutics.



Find a Clinical Trial

Easily search our family-friendly summaries of clinical trials and research studies.



PCORnet

DuchenneConnect is a member of PCORnet, the National Patient-Centered Clinical Research Network, an innovative initiative of the Patient-Centered Outcomes Research Institute (PCORI).



Duchenne Deletion Tool

Use this educational tool to help you understand if you/your child may be a candidate for an exon skipping therapy. This tool is only for individuals with a deletion of one or more exons in the Duchenne gene.

Exploring Clinical Trials

Clinical Trials Introductory Video

New to Learning about Clinical Trials?

- [Introductory Video](#)
- [What is a Clinical Trial?](#)
- [Types and Phases of Clinical Trials](#)
- [Clinical Trial Video Library](#)

New to clinical trials? Watch this short [introductory video](#) on some basics about clinical trials and what you and your family should consider.

Considering Trial Participation?

- [Clinical Trial Decision Guide](#)
- [Questions to Ask your Clinical Team](#)

Specific Trial Information

- [Find a Clinical Trial](#)



Clinical Trial Finder

Find a Clinical Trial

The following clinical trial and research study FAQ sheets are family-friendly summaries of clinical trials and research studies for people with Duchenne and Becker, as well as female carriers. There are also FAQ sheets for current pre-clinical research that is soon to be in clinical trial. These FAQ sheets are written for the program book of the PPMD Annual Connect Conference (held every June) in consultation directly with the Researchers. Although we try to include the majority of studies in the United States, this is not intended to be a comprehensive list.

Please note that while the [Antidote Clinical Trial finder tool](#) will also give you similar information there are some differences which are reviewed in the table below.

[Click here to list all FAQs by Title](#)

Studies by
Ambulation Status

Studies by
Mutation

Studies by
Corticosteroid Status

Ongoing and
Completed Studies

Studies for Female
Carriers

Preclinical Studies

Clinical Trial Finder

DuchenneConnect Home ▾ About DMD/BMD News ▾ Webinars & Media ▾ Clinical Trials ▾
Profile View Data Glossary Coordinators ▾

Non-Ambulatory Studies

Click any title to go to the

Studies by Ambulation

Studies by Mutation

Studies by Corticosteroid Use

Ongoing and Completed Studies

Studies for Female Carriers

DuchenneConnect Home ▾ About DMD/BMD News ▾ Webinars & Media ▾

Studies for Boys Currently on Corticosteroids

Last Updated December 18, 2017

Click any title to go to the study's FAQ page (when available). Click ClinicalTrials.gov next to title to go to the study's ClinicalTrials.gov

[Micro-dystrophin Gene Transfer in Adolescents and Children with Duchenne \(IGNITE DMD\) | ClinicalTrials.gov](#)
📍 [Gene Therapy](#) | Phase I/II | Open label, randomized, controlled, single ascending dose, safety and tolerability study | US | Recruiting
Age: 4 - 17 years **Amb. Status:** Ambulatory OR Non-Ambulatory **Steroid Status:** ≥ 6 months

[PF-06939926 - Mini-dystrophin Gene Therapy for Duchenne | ClinicalTrials.gov](#)
Gene Therapy | Phase Ib | Open label, non-randomized, ascending dose, safety and tolerability study | Infusion | Recruiting
Age: 5 - 12 years **Amb. Status:** Ambulatory **Steroid Status:** ≥ 6 months

Studies by Mutation
Studies by Corticosteroid Use
Ongoing and Completed Studies
Studies for Female Carriers

Clinical Trial Decision Guide

Clinical Trial Decision Guide

New to Learning about Clinical Trials?

- [Introductory Video](#)
- [What is a Clinical Trial?](#)
- [Types and Phases of Clinical Trials](#)
- [Clinical Trial Video Library](#)

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- [Questions to Ask your Clinical Team](#)

Specific Trial Information

- [Find a Clinical Trial](#)

These Decision Guides use interactive functions that are ONLY supported by the [Adobe Acrobat Reader®](#). Click the links below to download and save a copy of the zip file that contains the Adobe® PDF. You can then open them using your [Adobe Acrobat Reader®](#) software.

[>> Adult version of Clinical Trial Decision Guide <<](#)

[>> Pediatric version of Clinical Trial Decision Guide <<](#)

The PDF must be opened with [Adobe® Reader](#) or [Adobe® Acrobat](#) to be viewed correctly.

If you do not have [Adobe Reader®](#) or [Adobe® Acrobat®](#) installed on your computer, please install the free Adobe Reader from the trusted manufacturer website: <https://get.adobe.com/reader/>

Please note that the Decision Guides are not yet available for use on a smart phone.



MAKING THE RIGHT CHOICE ABOUT CLINICAL TRIALS
A Guide for Adults with Duchenne and Becker Muscular Dystrophy

Parent Project
Muscular Dystrophy
LEADING THE FIGHT TO END DUCHENNE

RTI
INTERNATIONAL

Helpful tips

- We recommend that you fill out the worksheet on your computer, save often, and then print a copy.
- Share it with those close to you and with your healthcare providers.
- Update it as needed.

IS A CLINICAL TRIAL RIGHT FOR ME?

Duchenne and Becker Clinical Trial Worksheet

Many people who join a clinical trial say they are satisfied and glad they participated. We want you to be satisfied with your decision, too. Thinking about what matters most to you can help you decide whether to join a trial or not. The goal of this worksheet is to help you think through your decision.

People will use this worksheet to make different choices. What are you thinking about when you fill out this worksheet?

- Being in trials in general
- One specific trial
- Choosing between two trials (you should fill out one sheet for each trial)

BRAINSTORM

What are some possible pros and cons of joining a clinical trial? Take a moment to list the things that matter most to you. Use one box for each pro or con you list. You can create as many boxes as you need.

Pros

Type your pro here.
Type your pro here.
Type your pro here.
Click to add another Pro

Cons

Type your con here.
Type your con here.
Type your con here.
Click to add another Con

Clinical Trial Video Library

Clinical Trial Video Library

New to Learning about Clinical Trials?

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- [What is a Clinical Trial?](#)
- [Types and Phases of Clinical Trials](#)
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- [Questions to Ask your Clinical Team](#)

Specific Trial Information

- [Find a Clinical Trial](#)

Click on each question below to view the video. If you haven't already watched our **introductory video** in [this video library](#), you may want to do so now.

How do I get the information I need to evaluate a clinical trial?

Families discuss the importance of understanding what types of questions to ask.

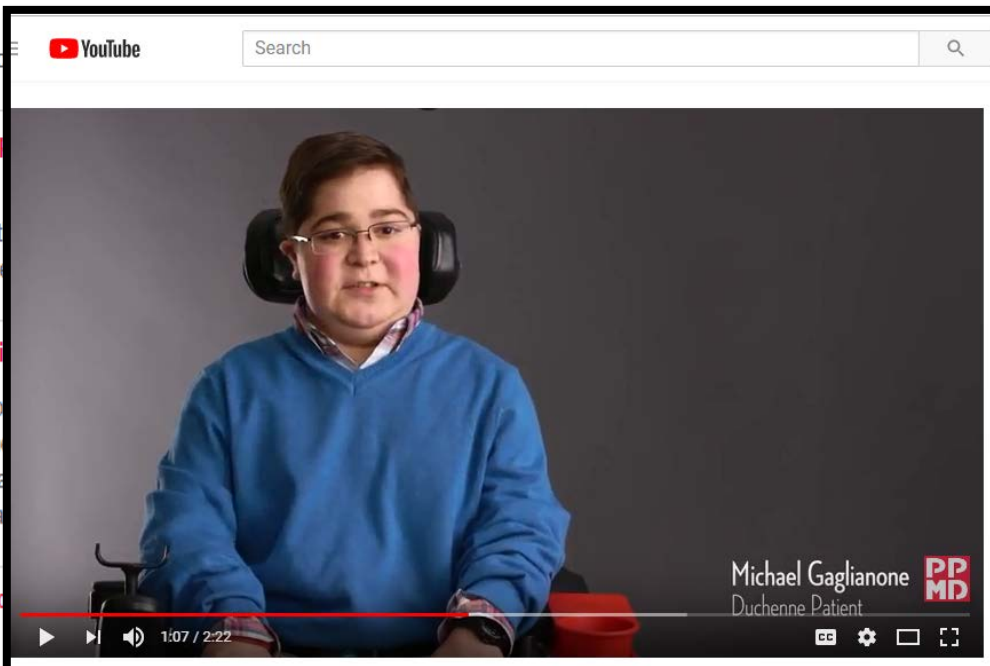
Why do we need a patient to be on it?

This video explores the importance of patient participation and what this means for people with Duchenne.

What are the benefits of clinical trials?

Clinical trials are explored in this video. While benefits of each trial. While benefits of clinical trials for families and health care providers, the benefits to being in a clinical trial are also discussed.

What are the risks of clinical trials for a child?



YouTube Search

Carrie Micelli, Ph.D.
Mother
Professor, Dept of Microbiology,
Immunology and Molecular Genetics
UCLA

PP MD

0:07 / 3:07

Benefits of Clinical Trials

This video player shows a woman with dark hair, Carrie Micelli, Ph.D., speaking. The video title is 'Benefits of Clinical Trials'. The player includes a search bar at the top, a progress bar at 0:07 / 3:07, and control icons for play, volume, and full screen. A red 'PP MD' logo is visible in the bottom right corner of the video frame.

YouTube Search

Kevin M. Flanigan, M.D.
Investigator at the Center for Gene Therapy
Nationwide Children's Hospital
Professor of Pediatrics and Neurology
Ohio State, University

0:04 / 3:25

Placebo Control

This video player shows a man with glasses, Kevin M. Flanigan, M.D., speaking. The video title is 'Placebo Control'. The player includes a search bar at the top, a progress bar at 0:04 / 3:25, and control icons for play, volume, and full screen.

Duchenne Deletion Tool

Exon deletion tool

If you know your/your child's genetic change (mutation) is an exon deletion, this educational tool can help you understand if you/your child may be a candidate for an exon skipping therapy. If you are unsure of your/your child's mutation, or if you are confused by your/your child's genetic test results you've received, please contact one of our genetic counselors to learn more.

Call 888-520-8675 or email coordinator@duchenneconnect.org

Instructions: Enter the first and last number correlating to your child's deletion in the fields below. If a single deletion, enter the same number in both fields.


Example: 12-12, 12-14, 12-75.

EXON 49	to	EXON 50	SUBMIT
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Duchenne Deletion Tool

Instructions: Enter the first and last number correlating to your child's deletion in the fields below. If a single deletion, enter the same number in both fields.

Example: 12-12, 12-14, 12-75.

EXON		EXON	
49	to	50	

Based on the information you entered, there may be a treatment option available for you/your child that skips **exon 51**. Talk to your local doctor or genetic counselor to learn more. You may also contact one of our genetic counselors at 888-520-8675 or coordinator@duchenneconnect.org.

- ▶ There may also be other **disease management options** and/or clinical trials available for you/your child. To learn more general information about clinical trials, visit PPMD's online [Clinical Trial Resource Center](#). For information on specific clinical trials, please visit DuchenneConnect's [FAQs](#) on clinical trials or visit ClinicalTrials.gov.

DuchenneConnect.org



coordinator@duchenneconnect.org

888-520-8675