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
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

Considering Trial Participation?
- Clinical Trial Decision Guide
- Questions to Ask your Clinical Team

Specific Trial Information
- Find a Clinical Trial

New to clinical trials? Watch this short introductory video on some basics about clinical trials and what you and your family should consider.
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
Clinical Trial Finder

Non-Ambulatory Studies

Click any title to go to the study.

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 page.

Micro-dystrophin Gene Transfer in Adolescents and Children with Duchenne (IGNITE DMD) | ClinicalTrials.gov
- Gene Therapy
- Phase 1/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
- US
- Recruiting
- Age: 5 - 12 years
- Amb. Status: Ambulatory
- Steroid Status: ≥ 6 months
Clinical Trial Decision Guide

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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
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.

<table>
<thead>
<tr>
<th>Pros</th>
<th>Cons</th>
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<tr>
<td>Type your pro here.</td>
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Click to add another Pro

Click to add another Con
Clinical Trial Video Library

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Click on each question below to view the video. If you haven’t already watched our introductory video into clinical trials, you may want to do so now.

How do I get the information I need to evaluate a clinical trial?
Families discuss the questions they ask. What types of questions do they ask? Why do we need a model to be on it?

What are the benefits?
Clinical trials are expensive, and each trial. While benefits to families and health care professionals, benefits to being in a clinical trial include...

What are the risks of participation?

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
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.

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.