

PPMD “Living Duchenne” Resource Center

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Parent JOIN THE FIGHT.
Project END DUCHENNE.
Muscular
Dystrophy

The Need

- Scattered information on resources
- Social media groups (buried detail)
- State specific needs
- Where do you start?
- What can you trust?



Living Duchenne Resource Center

The screenshot shows a web browser window displaying the 'PPMD Community Resource Center'. The main heading is 'PPMD Community Resource Center' with a subtext: 'Collaborate with other members to build resources for the Duchenne community.' Below this is a large graphic of a hand holding a red pen writing 'DUCHENNE COLLABORATIVE RESOURCES' on a document. To the right of the main content is a sidebar with navigation links: 'Sign Out', 'Inbox (7 new)', 'Alerts', 'Friends (1 request)', and 'Settings'. Below these are sections for 'AWAITING APPROVAL' (5 New Members), a help link 'Need help using this community site? Visit Ning's Help Page.', social media icons for Facebook, Twitter, and YouTube, a 'MEMBERS' section with a grid of profile pictures, and an 'EVENTS' section for January 2018. At the bottom of the main content area, there is a paragraph: 'The PPMD community resource center is meant to be a one-stop resource for every child, adult, and family living with Duchenne. Here you can find some of the favorite resources, products, and organizations of our community members.' followed by a call to action: 'As a community resource, we need your help to develop this center in order to most effectively reflect your needs.'

PPMD Community Resource Center

Collaborate with other members to build resources for the Duchenne community.

DUCHENNE COLLABORATIVE RESOURCES

Sign Out

Inbox (7 new)

Alerts

Friends (1 request)

Settings

AWAITING APPROVAL

5 New Members

Need help using this community site? Visit [Ning's Help Page](#).

MEMBERS

EVENTS

January 2018

The PPMD community resource center is meant to be a one-stop resource for every child, adult, and family living with Duchenne. Here you can find some of the favorite resources, products, and organizations of our community members.

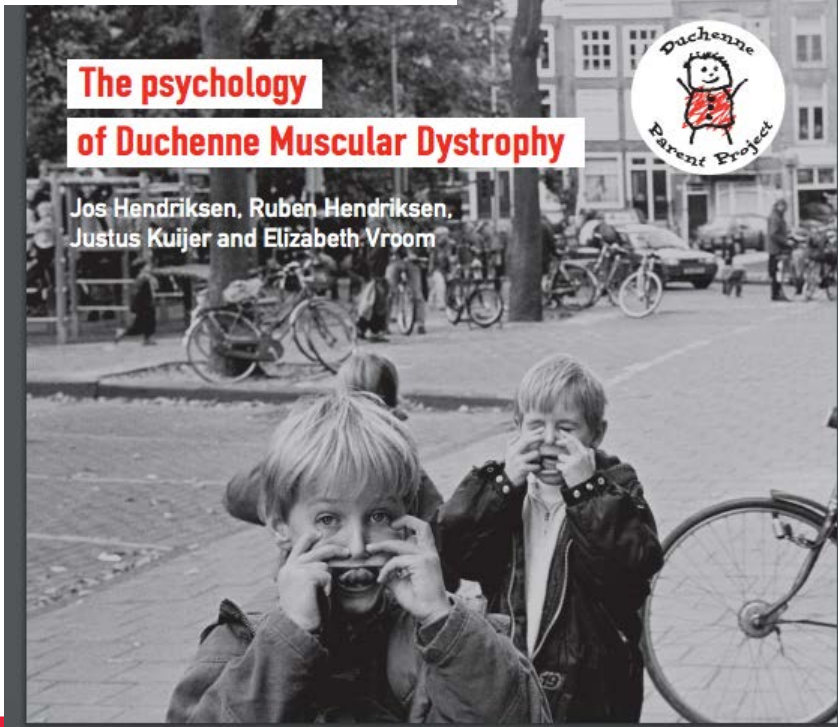
As a community resource, we need your help to develop this center in order to most effectively reflect your needs.

www.parentprojectmd.org/resources

Categories

Education Section

A Teacher's Guide to Duchenne (Education Matters)



- Education Matters
- The psychology of Duchenne
- Adaptive PE
- Behavior and Duchenne
- School Resources for class education

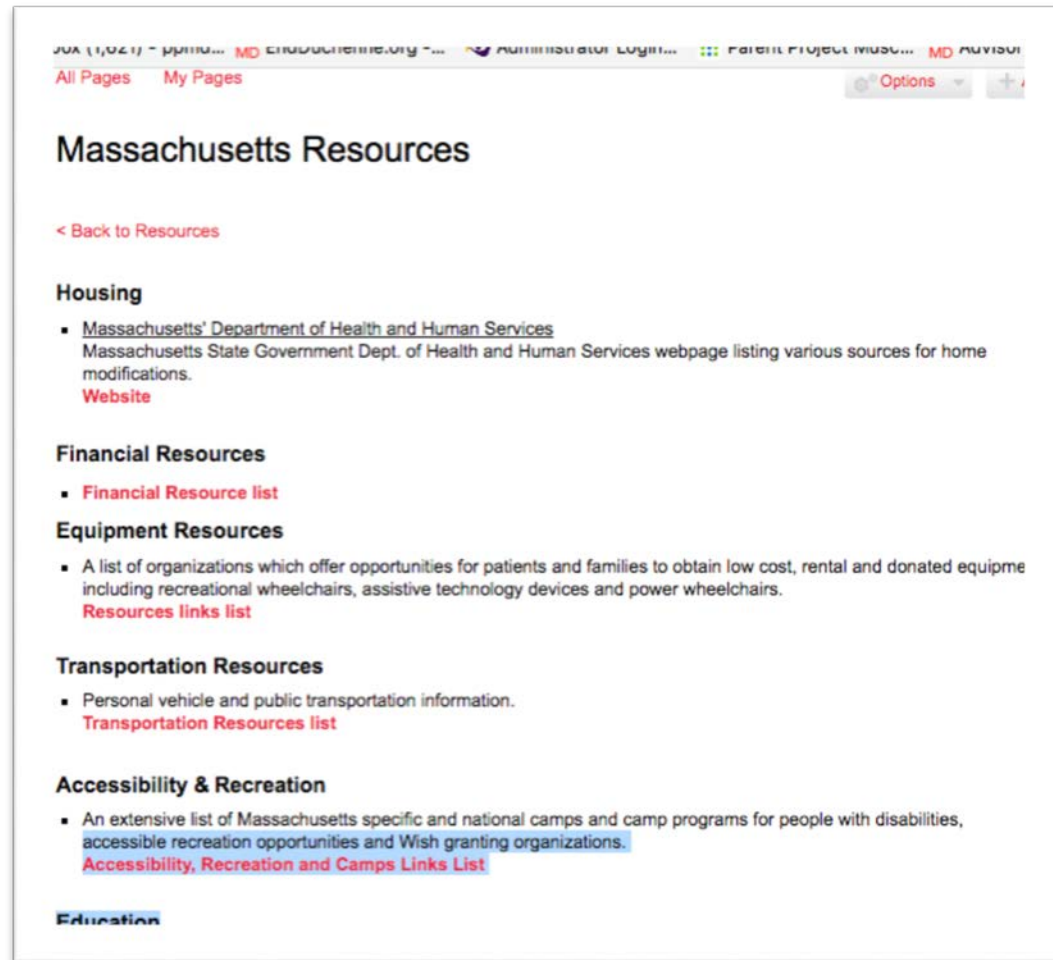
PPMD Connect Program

- Established 2008 formally called FACES
- The official parent led outreach initiative of PPMD
- 24 regional groups around the country
- Focus on connecting, resource sharing, support, and advocacy (grassroots army!)
- New manager of program – Nicole Herring
- PPMD State Resource Effort
- Many work with clinics within their states

24 States specific groups established

- **New Jersey**
- **Illinois**
- **Arizona**
- **South Texas**
- **Colorado/Wyoming**
- **North Texas**
- **Pacific North West**
- **Michigan**
- **Northern California**
- **Western New York**
- **Ohio**
- **Southern California**
- **Tennessee**
- **Florida**
- **Nebraska**
- **Virginia**
- **Northern Florida**
- **North Carolina**
- **Georgia**
- **Minnesota**
- **DMV - DC, Maryland, Northern VA**
- **Western Pennsylvania**
- **Long Island New York**

State Specific Page Example



The screenshot shows a web browser window with the following content:

Browser tabs: JWA (1,021) - p... MD EndDuchenne.org - ... Administrator Login... Parent Project Muscu... MD Advisor

Page navigation: All Pages My Pages Options

Massachusetts Resources

[< Back to Resources](#)

Housing

- [Massachusetts' Department of Health and Human Services](#)
Massachusetts State Government Dept. of Health and Human Services webpage listing various sources for home modifications.
[Website](#)

Financial Resources

- [Financial Resource list](#)

Equipment Resources

- A list of organizations which offer opportunities for patients and families to obtain low cost, rental and donated equipment including recreational wheelchairs, assistive technology devices and power wheelchairs.
[Resources links list](#)

Transportation Resources

- Personal vehicle and public transportation information.
[Transportation Resources list](#)

Accessibility & Recreation

- An extensive list of Massachusetts specific and national camps and camp programs for people with disabilities, accessible recreation opportunities and Wish granting organizations.
[Accessibility, Recreation and Camps Links List](#)

Education

Access to approved therapies resource page

www.parentprojectmd.org/accessresources



Understanding the Path to Access – Resources for the Community

Posted by PPMD on December 19, 2016 at 12:30pm

[View Blog](#)



Goals

Provide education and guidance on access process

Provide product specific resources for Patients, Clinicians, Payers

Understanding Access Stages

STAGE 1

Prescription written by
Doctor and submitted to
Insurer



STAGE 2

Patient, Drug Company,
and Prescribing Doctor
work with Insurer to
process prescription



STAGE 3

Drug is provided to
Patient

STAGE 1

Prescription written by
Doctor and submitted to
Insurer

- To begin the process for access, most drug companies have a **start form** that can be filled out by you or your physician.
- Some insurers will require what's called **Prior Authorization**. This may involve confirmation of genetic test results and other medical records to be submitted with the **prescription from your doctor**.
- **Your clinician will be a key advocate for this process.** He/she should consider submitting a **Letter of Medical Necessity** (sample letter below), which provides background information about the patient, disease, and drug

EMFLAZA

STAGE 1

Prescription written by
Doctor and submitted to
Insurer

Emflaza is a corticosteroid that demonstrates anti-inflammatory and immunosuppressant effects. Emflaza is available in an immediate-release tablet formulation at multiple dosages (6 mg, 18 mg, 30 mg, 36 mg) as well as in an oral suspension formulation (22.75 mg/mL).

Please contact [PTCCares](#) to be connected with a case manager who can help you begin the process for access. [PTCCares](#) will contact your physician for the [start form](#) and the prescription.

[Emflaza label and prescribing information](#)

[Emflaza FAQs](#)

[Preparing for Access to Approved Therapies](#)

Learn about the [AssistanceFund](#)

EXONDYS 51

STAGE 1

Prescription written by
Doctor and submitted to
Insurer

Unsure if you are amenable to this therapy?

Try our new [Deletion tool](#) to perform a search on your mutation

Visit [PPMD's DuchenneConnect registry](#) to learn about your genetic mutation or access free genetic testing.

For U.S. Residents

For those amenable to exon 51 skipping please contact [SareptAssist](#) to be connected with a case manager who can help you begin the process for access. [SareptAssist](#) will contact your physician for the start form and the prescription.

Learn about the [AssistanceFund](#)

[Sample Letter of Medical Necessity \(for Clinicians/Doctors\)](#)

[FDA label document](#)

[SareptAssist Patient Services Overview \[PPMD Webinar Recording\]](#)

If you live outside of the U.S.

Sarepta supports a Managed Access Program (Expanded Access Program) for those residing in numerous countries, including: Argentina, Brazil, Canada, Colombia, France, Germany, Greece, Iceland, Italy, Mexico, Spain, Turkey, and the United Kingdom.

For more information about eligibility and access, visit Sarepta's Managed Access Program [by click here](#).

The landscape of Duchenne is rapidly changing!

New therapies are being developed, some of which will help any person with Duchenne, while others depend on the person's genetic change, or mutation. Knowing the mutation is important, as it can affect decision-making about the possible use of new therapies and entering into clinical trials.

Duchenne is caused by changes (mutations) within the dystrophin gene. A gene is made up of coding regions call "exons," and the areas in between exons called "introns." Dystrophin has 79 exons, which makes it the largest gene in the body.

Genetic testing can identify your/your child's specific mutation. If you/your child need genetic testing, please talk with your doctor, genetic counselor, or one of the **DuchenneConnect genetic counselors.**

New tool for families and professionals

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
ENTER
FIRST EXON
DELETED

to

EXON
ENTER
LAST EXON
DELETED

SUBMIT

STAGE 2

Patient, Drug Company,
and Prescribing Doctor
work with Insurer to
process prescription

What happens if access is denied by my insurer?

Things to Consider:

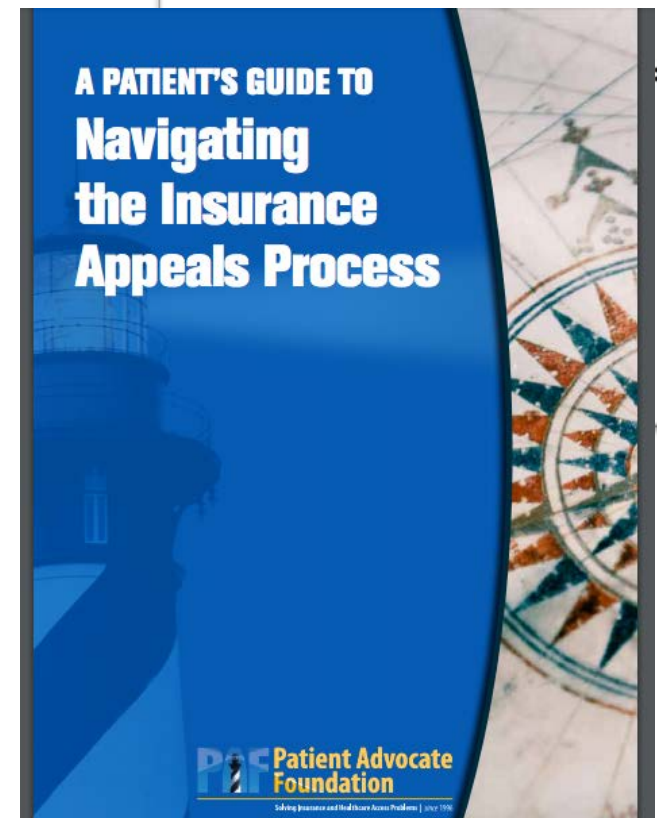
- ✓ Ask your clinician and the insurer about a "peer to peer" with payer
- ✓ Write your own **Patient Appeal Letter**
- ✓ Has your clinician submitted a **Letter of Medical Necessity**?

Family Resources

- ✓ Letter appeal templates
- ✓ Supported documentation about benefit
- ✓ Contact information for PTC Cares and Sareptassist

Tips and resources

- **Track all communications** with the drug company, health insurer, and clinician/doctor.
- **Consider contacting your state Consumer Assistance Program (CAP)** or Department of Insurance during an appeal process. Visit localhelp.healthcare.gov.
- **Get familiar with your health insurer's policy.**
- **You have the right for an Appeal.** Your insurer must outline what that appeals process entails and specifically why you were turned down.
- **You CAN request an urgent or expedited appeal** if your medical provider believes a delay in treatment could jeopardize your overall health.



We need your help!

- Clinics provide families with resources and information regularly
- Specific resources about your clinic?
- Resources you share?
- How can PPMD be more helpful to clinics?
- Goal to continuing building this resource and make it as robust as possible

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