PPMD “Living Duchenne” Resource Center

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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

www.parentprojectmd.org/resources
Categories
Education Section

A Teacher’s Guide to Duchenne (Education Matters)

The psychology of Duchenne Muscular Dystrophy

- 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
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

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
• 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 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

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 called "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.

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.
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](http://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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