

**THANK YOU TO OUR SPONSORS FOR
YOUR SUPPORT OF THIS MEETING**

**Parent Project
Muscular
Dystrophy** JOIN THE FIGHT.
END DUCHENNE.



**Parent
Project
Muscular
Dystrophy**

END DUCHENNE TOUR



Chicago, IL

Impact on Care



10
years

added to average
lifespan due to
PPMD-led advances



38
clinics

awarded certification by
PPMD across the US as
of February 2023



750 care
providers

in PPMD network of
certified clinics

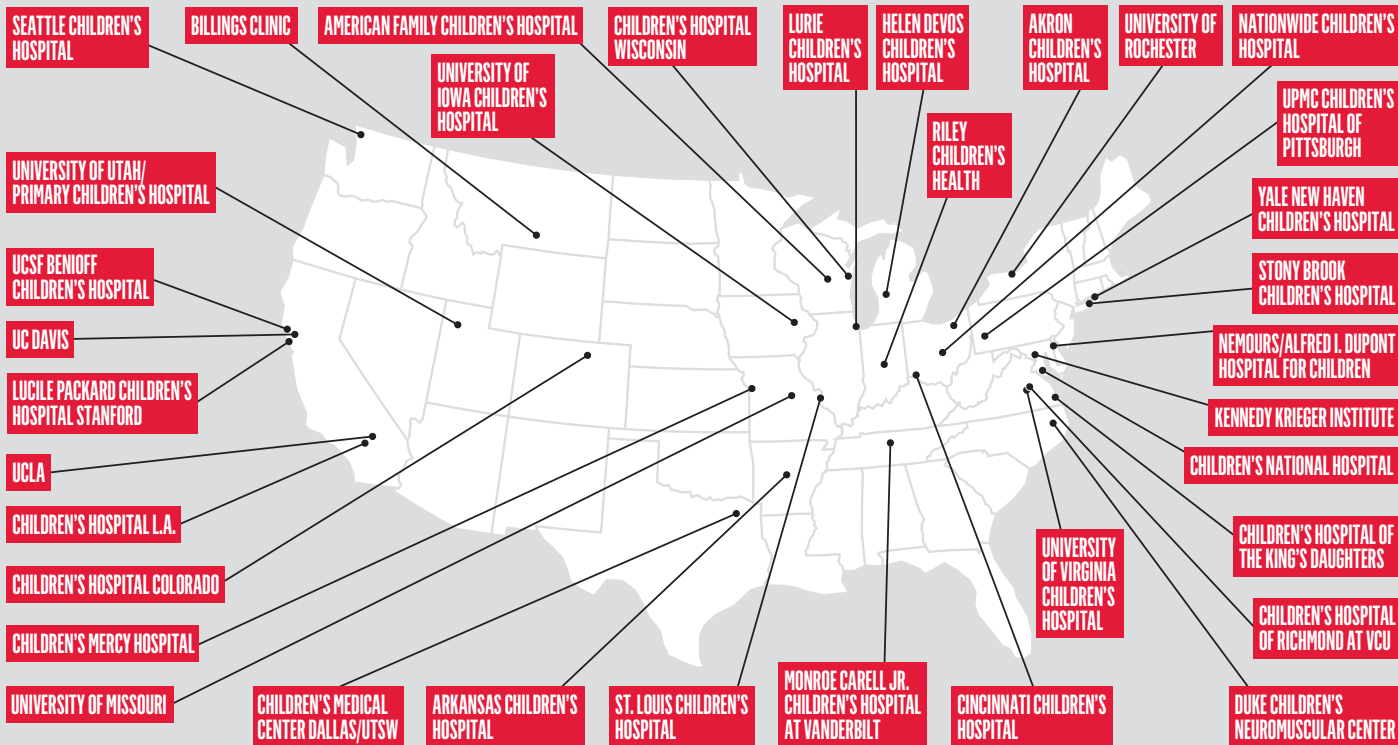


20
years

of identifying & addressing
gaps in care through
specialty workshops &
consensus meetings

PPMD's Certified Duchenne Care Centers

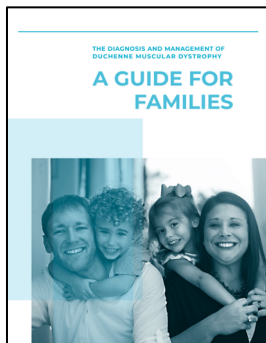
Parent
Project
Muscular
Dystrophy



Plus, two Global Certified Duchenne Care Centers: Red Cross War Memorial Children's Hospital (South Africa) and Motol University Hospital in (Czech Republic)

*As of November 12, 2022

DUCHENNE CARE GUIDES



FAMILY CARE GUIDE

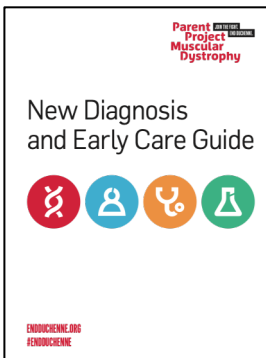
Partnered with MDA, Treat-NMD, and WDOIncludes an overview of the Duchenne Care

Considerations across the lifespan in an “easier-to-digest” format



RANGE OF MOTION GUIDE

PPMD worked with a team of expert Physical Therapists (PTs) to develop an easy-to-follow guide for parents and physical therapists.



NEW DIAGNOSIS & EARLY CARE GUIDE

“Duchenne 101” and genetic testing, adjusting to the diagnosis and finding support, early care, and introduction to clinical trials



DUCHENNE CARRIERS: YOUR QUESTIONS ANSWERED

This comprehensive guide addresses the questions most often asked by carriers.

Developed in collaboration with carriers and with expert providers from neurology, genetics, cardiology, and psychology

Care & Support Materials



CARE CONSIDERATIONS CHECKLIST
EARLY AMBULATORY STAGE/CHILDHOOD

Parent Project Muscular Dystrophy

This checklist is intended for use by parents and caregivers of individuals with Duchenne muscular dystrophy to help you manage your child's care. Not all people with Duchenne will need to see a specialist in each medical area below. Talk with your child's neuromuscular specialist about any concerns and referrals. At this stage, children are usually showing signs of Duchenne. Use a walking type of aids, walking on their toes, or needing to support themselves with their hands when they get up from the floor (called a Gower's maneuver). Children with Duchenne may also have speech and/or other development delays.

Neuromuscular Care
 Your child's neuromuscular specialist (NMS) is the lead doctor for your child's care, who specializes in muscle care. The NMS will help you and your family understand Duchenne and the special care for your child. You should see the NMS about every 6 months.

- The NMS, along with the rehabilitation team, measures your child's function, strength, range of motion, posture, and positioning of arms and legs using the same measures and tools at each visit. This evaluation is done every 6 months or as needed.
- Make sure your child is up-to-date on all immunizations. This website has information on immunizations for individuals with Duchenne: ParentProjectMD.org/Vaccinations.
- Discuss the benefits and side effects of corticosteroids (steroids), such as prednisone or deflazacort (also known as Emflaza), and other appropriate therapies with the NMS as early as possible. Consider starting steroids before your child starts to lose muscle function, as steroids are most effective when used as the disease begins.
- Never start taking steroids abruptly.
- The NMS will send a summary of each visit to you, your child's primary care provider (PCP), and communicate with other providers as needed.

Cardiac Care
 A cardiologist is a doctor who specializes in the heart who you will see every year.

- Your child's heart health should be evaluated every year using an electrocardiogram (ECG) and an echocardiogram or cardiac MRI (magnetic resonance imaging). These show your child's heart rate, rhythm, structure, and function.
- Start taking heart medication when cardiac tests show a decline in heart function or warning (fibrosis) of the heart muscle, or by age 10 even if all tests are normal.

Respiratory Care
 A pulmonologist is a doctor who specializes in the lungs who you will see every year.

- A pulmonologist will have regular outpatient pulmonary function testing (PFT) starting at the time of diagnosis or by age 5. This will get your child used to doing the test, and will allow your pulmonologist to track the strength of muscles responsible for breathing over time.
- Ensure your child receives the appropriate flu vaccine every year and pneumococcal vaccine as recommended.
- Your child's pulmonologist may suggest a cough study if there are signs and symptoms of shallow breathing, called hypoventilation, while sleeping.
- Your child's pulmonologist may suggest a sleep study if there are signs and symptoms of shallow breathing, called hypoventilation, while sleeping.

Notes

COMMON LABS AND WHAT THEY MEAN

Parent Project Muscular Dystrophy

NEW RESOURCE!

COMMON LABS & WHAT THEY MEAN IN DUCHENNE

The following are labs that are commonly done in neuromuscular centers. Normal ranges have been provided for medlineplus.gov. Current versions for obtaining these labs as well as desirable reasons for high and low values, as well as any cautions, are included. This list is not exhaustive and any abnormal values, questions, or concerns should be discussed with your neuromuscular specialist.

Blood Test	Normal Range	Abnormal Common Reasons (Not ALL reasons for the Outside of Normal Range)	Notes
Complete Blood Count (CBC)			
White Blood Cell Count (WBC)	5,000-10,000 WBCs per mm ³ or 5.0-10.0 x 10 ⁹ WBC per liter	Abnormal Common Reasons (Not ALL reasons for the Outside of Normal Range): - CBC includes components such as white blood cells (leukocytes), hemoglobin, and hematocrit. An elevated WBC count may indicate an infection or inflammation. - High. Chronic inflammation may be associated with several conditions. - Low. Anemia	
Red Blood Cell Count (RBC)	Male: 4.5-5.5 million RBC per mm ³ or 4.5-5.5 x 10 ¹² RBC per liter Female: 4.0-5.0 million RBC per mm ³ or 4.0-5.0 x 10 ¹² RBC per liter	Abnormal Common Reasons (Not ALL reasons for the Outside of Normal Range): - Low. Anemia	
Hemoglobin (Hgb)	Male: 14-16 g/dL or 140-160 g/L Female: 12-15 g/dL or 120-150 g/L	Abnormal Common Reasons (Not ALL reasons for the Outside of Normal Range): - Low. Anemia	
Hematocrit (Hct)	Male: 40-50% or 0.40-0.50 Female: 37-47% or 0.37-0.47	Abnormal Common Reasons (Not ALL reasons for the Outside of Normal Range): - Low. Anemia	
Mean Corpuscular Volume (MCV)	80-100 fL or 80-100 fL	Abnormal Common Reasons (Not ALL reasons for the Outside of Normal Range): - High. Macrocytic anemia - Low. Microcytic anemia	
Mean Corpuscular Hemoglobin (MCH)	27-34 pg or 27-34 pg	Abnormal Common Reasons (Not ALL reasons for the Outside of Normal Range): - High. Macrocytic anemia - Low. Microcytic anemia	
Mean Corpuscular Hemoglobin Concentration (MCHC)	32-36 g/dL or 32-36 g/dL	Abnormal Common Reasons (Not ALL reasons for the Outside of Normal Range): - High. Macrocytic anemia - Low. Microcytic anemia	

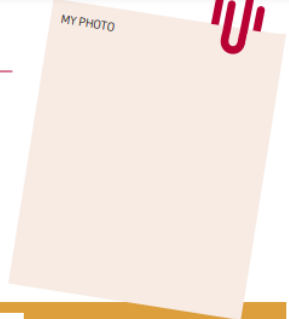
MCV, MCH, and MCHC are used to determine the average size of hemoglobin in red blood cells.
 Low MCV indicates anemia.
 High MCHC indicates anemia.

Resources for School



HI! MY NAME IS:

I have Duchenne muscular dystrophy. This is a rare genetic disorder that makes my muscles progressively weaker over time. Duchenne is something I was born with and is not contagious. Duchenne also affects other systems in my body including my heart and lungs, and sometimes the way I learn & behave. Because I have Duchenne, I may get tired more easily or need help with certain things. If you see me struggling, please ask how you can help! There are a few other things I want you to know about me so we can have the best school year:



LIKES

STRENGTHS

CHALLENGES

WHAT WORKS FOR ME

To learn more about Duchenne visit parentprojectmd.org



**IN AN EMERGENCY
REMEMBER TO**

THINK

ParentProjectMD.org/think

T

**TAKE YOUR EQUIPMENT
WITH YOU TO THE HOSPITAL**

H

**HAND YOUR EMERGENCY
CARE CARD TO EMERGENCY
MEDICAL TEAM TO REVIEW**

I

**INFORM MEDICAL STAFF
OF IMPORTANT OXYGEN
PRECAUTIONS**

N

**NOTIFY YOUR
NEUROMUSCULAR TEAM**

K

**KEEP IMPORTANT
DOCUMENTS WITH YOU**



EMERGENCY

C CONTACTS

A ALLERGIES AND PRECAUTIONS

R REVIEW MEDICATIONS AND MEDICAL CONDITIONS

E EMERGENCY CARE INFORMATION

Duchenne/Becker muscular dystrophy (Duchenne) is a rare genetic disorder that causes progressive muscle weakness over time. It affects every system in the body including skeletal muscle, heart, lungs, and brain.

Duchenne affects primarily boys (about 1 in 5,000 boys). Each year around 20,000 babies worldwide are born with Duchenne.

There is no cure for Duchenne, however there are many supportive cares that can be taken to improve outcome and quality of life.

STUDENT INFORMATION

Name: M F D.O.B. _____

Address: _____

PERSONAL:

Name: _____ Name: _____

Relation: _____ Relation: _____

Phone: _____ Phone: _____

Address: _____ Address: _____

Medical:

Neuromuscular Specialist/Hospital: _____ Primary Care Provider: _____

Phone: _____ Phone: _____

After Hours Phone: _____ After Hours Phone: _____

Allergies:

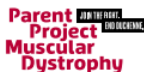
DO NOT GIVE THESE MEDICATIONS

- Succinylcholine
- Inhaled anesthetics
- Oxygen should only be administered with close CO2 monitoring or pressure support
- Other: _____

Medications:

- | | |
|--|--|
| <input type="checkbox"/> Duchenne Muscular Dystrophy | <input type="checkbox"/> Pacemaker/ICD |
| <input type="checkbox"/> Obstructive Sleep Apnea | <input type="checkbox"/> Visually Impaired |
| <input type="checkbox"/> Cardiomyopathy | <input type="checkbox"/> Hearing Impaired |
| <input type="checkbox"/> Adrenal Insufficiency | <input type="checkbox"/> High Blood Pressure |
| <input type="checkbox"/> Asthma | <input type="checkbox"/> Kidney Disease |
| <input type="checkbox"/> Diabetes | <input type="checkbox"/> Malignant Hypothermia |
| <input type="checkbox"/> Autism | <input type="checkbox"/> Seizure Disorder |
| <input type="checkbox"/> Cognitive Delay | <input type="checkbox"/> Other: _____ |

Scan the QR code or visit parentprojectmd.org/emergencycare



DUCHENNE MUSCULAR DYSTROPHY EMERGENCY CARE INFORMATION FOR FAMILIES



RESPIRATORY CARE

Follow your child's pulmonary action plan! If trouble breathing, or Oxygen saturation low, use cough assist or Ambu bag or BiPAP to clear the airway. If breathing does not improve in 5-10 minutes, go to ER. **Bring all equipment and medications with you to the Emergency Room (ER) if possible.**

LEG FRACTURE TREATMENT

If your child has leg pain following a fall, go to Urgent Care or ER to get an X-ray. **If your child has difficulty breathing, seems confused, or is less alert after a fall/fracture, this is an emergency; go immediately to the ER and alert staff that symptoms could be due to Fat Embolism Syndrome (FES).**

NEUROMUSCULAR CENTER/DOCTOR: _____

STEROIDS

Remember to tell your doctor if your child is on steroids. If severe trauma or unable to take daily corticosteroids for 48 hours, go to the ER and ask that IV corticosteroids are given until pills by mouth are tolerated (**6 mg of deflazacort equals 5 mg of Prednisone**). Bring the PJ Nicholoff Steroid Protocol (parentprojectmd.org/pj). Stress doses may be needed for moderately severe stress on the body.

ANESTHESIA PRECAUTIONS

If possible, inhaled anesthesia should be avoided. IV anesthesia is considered safe with close monitoring. **Succinylcholine should NEVER be used.** Local anesthesia and nitrous oxide are generally safe for minor dental procedures.

GENERAL RECOMMENDATIONS

- Keep immunization up to date & get influenza (flu) vaccine annually.
- Always wear seat belts in the car AND in chairs/wheelchair/scooter/shower chairs.
- Call your neuromuscular team and tell them you are going to the ER/hospital (do not depend on the ER staff to do this).

NEUROMUSCULAR CENTER EMERGENCY NUMBER: _____

DUCHENNE MUSCULAR DYSTROPHY EMERGENCY CARE INFORMATION FOR HEALTHCARE PROVIDERS



Parent Project Muscular Dystrophy

RESPIRATORY CARE

Risk of respiratory failure. **Do not give Oxygen** without close monitoring of CO2 levels. Breathing may need to be supported (non-invasive ventilation). Use cough assist machine if needed and available.

LEG FRACTURE TREATMENT

Risk of pain, loss of ambulation, FES. If ambulatory before leg fracture, surgery is preferred over casting to preserve ambulation (i.e. internal fixation with rapid weight bearing). Following a fracture or body trauma, **watch for signs of Fat Embolism Syndrome (FES)** including fast breathing and/or confusion.

STEROIDS

Risk of adrenal crisis. Please refer to the PJ Nicholoff Steroid Protocol (parentprojectmd.org/pj) for stress dosing. Watch for signs of adrenal crisis during times of severe illness, injury, or surgery.

ANESTHESIA PRECAUTIONS

Risk of rhabdomyolysis. Inhaled anesthesia can cause rhabdomyolysis among other serious complications (i.e. cardiac arrest) in patients with Duchenne. When possible, should anesthesia should be avoided. IV anesthesia is considered safe. Use all anesthesia with extreme caution after discussing with the anesthesiologist. **Succinylcholine should never be used.** Local anesthesia and nitrous oxide are generally safe for minor dental procedures.

GENERAL RECOMMENDATIONS

- Consider long term steroid therapy when administering live vaccinations.
- ACTAAE are normally elevated in patients with Duchenne and need no further evaluation.

PJ Nicholoff Steroid Protocol

About this Document

This document is meant for healthcare providers. For further explanation of steroids and adrenal crisis, please see the PPMO Steroid Care Page at parentprojectmd.org/steroids.

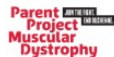
Background/Assessment

Normal basal secretion of cortisol from the adrenal gland is approximately 5-7 mg/daily or 8-10 mg/day for adults. This amount increases during minor illnesses or surgery to approximately 50 mg/day (5x normal physiologic secretion). These small increases with uncomplicated surgery return to baseline in 24 hours. Procedures producing greater surgical stress, have been shown to increase cortisol responses to 75-150 mg/day (10x normal physiologic secretion), which return to baseline in about 5 days.

Corticosteroids are prescribed for multiple diagnoses to a wide variety of patients. Long term administration of corticosteroids may lead to suppression of the hypothalamic-pituitary-adrenal (HPA) axis. Rapid reduction or abrupt withdrawal of corticosteroid therapy that has been prolonged or at high doses can cause secondary adrenal insufficiency (suppression of the HPA axis), and steroid withdrawal or deprivation syndrome. Recovery from suppression of the HPA axis after discontinuing corticosteroids can be prolonged (possibly 6 to 12 months) and may vary based on doses, dosing schedules and duration of corticosteroid therapy. Since there is a great deal of individual variability in susceptibility to suppression of the HPA axis after chronic use of exogenous corticosteroids, it is not possible to predict with confidence which patients will be affected. Current practice is to administer supplemental (stress) doses of corticosteroids to patients with suspected suppression of the HPA axis in the preoperative period and during acute illness to prevent acute adrenal insufficiency, or adrenal crisis.

Defining HPA Suppressed Patients:

Recommendations differ slightly in defining a suppressed patient, but general guidelines are below (Table 1):



FOR HEALTHCARE PROVIDERS



One-to-one meetings with the PPMD team for personalized support.



parentprojectmd.org/foryou

New Diagnosis

Schedule this meeting type if you have received a recent diagnosis of Duchenne or Becker in the past 2 years. We are here to discuss what this means, what you can e...

Genetic Counseling, Trials & The Duchenne Registry

Meet with one of PPMD's Certified Genetic Counselors to ask questions about your child's genetics, carrier or reproduction questions, discuss The Duchenne Registry ...

Life Coaching

Schedule this meeting to discuss one on one with Pat Moeschel, PPMD Director of Transition, on all things life and mental health related for those living with Duche...

Connect with the Community

Being able to connect with other families who have been through this Duchenne journey before can be extremely helpful and comforting, knowing you are not alone. Sch...

Care Coordination

Schedule this meeting type for questions regarding PPMD's Certified Duchenne Care Center (CDCC) program, for questions about a particular area of care, or for help ...

Navigating Resources and Benefits

Schedule this meeting type if you are looking for care and support resources, or to discuss navigating benefits or access programs.

Advocacy

Schedule this meeting to talk with a member of PPMD's Advocacy team to see how you can get involved in advocacy efforts at the state and federal level. For more i...

DIY Fundraising

Want to start a fundraiser but not sure where to begin? Schedule this meeting to talk with a member of PPMD's staff to discuss steps to setting up your own fundrais...

School & Education

Schedule this meeting type if you have questions regarding talking to your child's school and planning for IEP meetings. We can also provide resources to make your ...

Facilitating Independence

Schedule this meeting if you would like to discuss living independently, navigating college/campus life, finding a job, etc. for yourself or your child with Duchenn...

Race to End Duchenne

Schedule this meeting to discuss PPMD's Race to End Duchenne program or other fundraising strategies. Email jamie@parentprojectmd.org for more information

Ways to Engage with the Duchenne Community

- Family & Adult Outreach Programs
- Annual Conference
- Endurance Fundraising
- DIY Fundraising
- Coach to Cure MD
- Advocacy





PPMD's Connect

- Volunteer-led family outreach groups
- 28 across the country
- In-person and virtual family get-togethers
- Facebook groups
- Great way for families to find support and build a network of connection



Grandparents and Siblings

- Grandparents & Extended Family Members Group
 - Meets virtually every other month
 - Speakers cover relevant topics
 - Time for connection and Q&A



- Sibs Connect
 - Facebook group for 16+
 - Virtual socials
 - Conference sessions



Newly Diagnosed Program

- Annual Conference scholarship
 - Dedicated sessions just for new families
 - Mentorship program
 - Free conference registration
- Materials
 - Newly Diagnosed Families Guide
 - Education Matters
 - Dedicated section of website
 - Free resource center
- Meet & Greets (virtual) throughout the year. Contact Nicole@parentprojectmd.org for details about these sessions.

PPMD's Adult Advisory Committee (PAAC) & Tween Group

- Represent the teen and adult voice of people living with Duchenne and Becker
- Serve as leaders in the community and actively participate in:
 - PPMD federal advocacy efforts
 - Industry-led focus groups
 - Duchenne adult-specific care programs and resources
 - Awareness projects
 - Mentorship and social connection
- Tween Socials – virtual, quarterly get togethers for 9-13 year olds living with Duchenne





2024 Annual Conference *30th Anniversary*

- June 27-30 in Orlando, FL
- To celebrate our 30th anniversary, we are offering families a special \$30 registration fee
- Largest, most comprehensive annual international conference focused on Duchenne & Becker
- Attended by families, physicians, researchers, caregivers, industry partners and those living with Duchenne
- Kids Track while parents attend sessions
- Includes sessions for grandparents, siblings, dads