THANK YOU TO OUR SPONSORS FOR YOUR SUPPORT OF THIS MEETING

Parent Project Muscular Dystrophy
JOIN THE FIGHT. END DUCHENNE.

AVIDITY BIOSCIENCES

Edgewise THERAPEUTICS

entrada THERAPEUTICS

ITALFARMACO

NS Pharma

Pfizer

PTC THERAPEUTICS™

REGENXBIO®

santhera
THEIR FUTURE - OUR FOCUS

SAREPTA THERAPEUTICS

Parent Project Muscular Dystrophy

END DUCHENNE TOUR

Chicago, IL

Impact on Care

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10 years

added to average lifespan due to PPMD-led advances

38 clinics

awarded certification by PPMD across the US as of February 2023

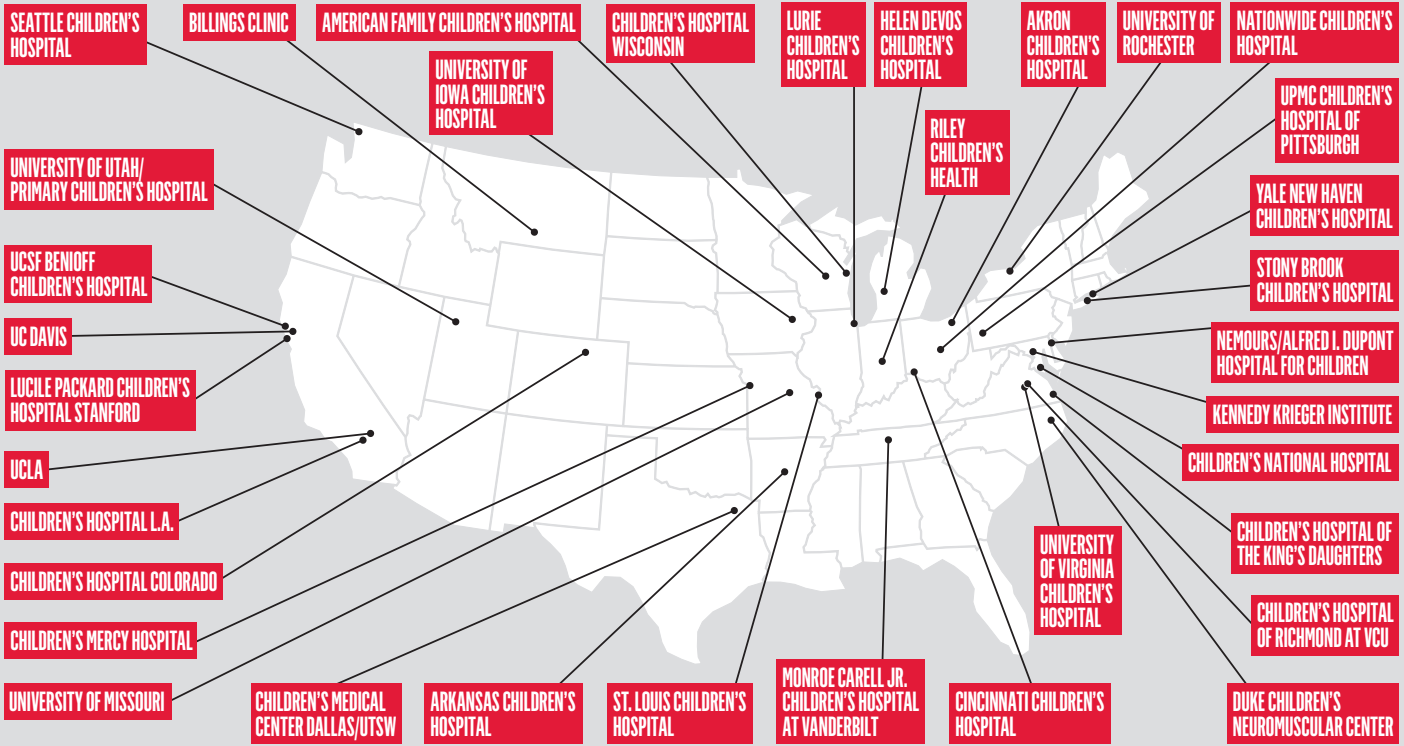
750 care providers

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



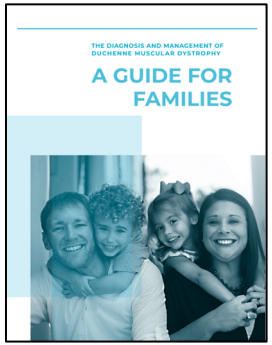
Plus, two Global Certified Duchenne Care Centers: Red Cross War Memorial Children's Hospital (South Africa) and Motol University Hospital in (Czech Republic)

*As of November 12, 2022

Parent Project Muscular Dystrophy

DUCHENNE CARE GUIDES

CARE | Parent Project Muscular Dystrophy — JOIN THE FIGHT. END DUCHENNE



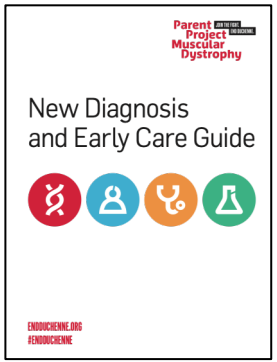
FAMILY CARE GUIDE

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

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

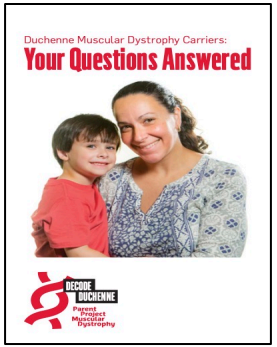
RANGE OF MOTION GUIDE

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



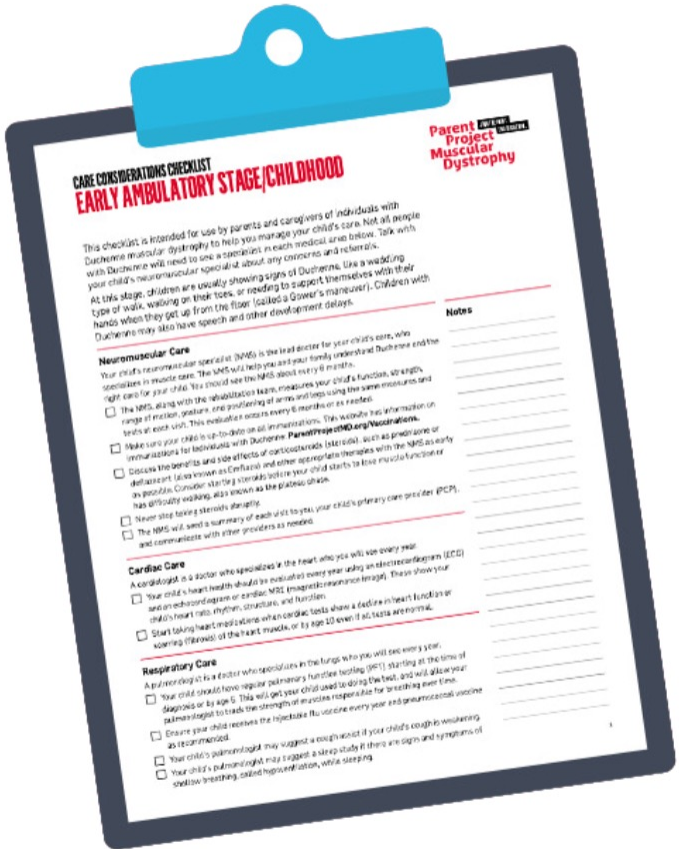
DUCHENNE CARRIERS: YOUR QUESTIONS ANSWERED

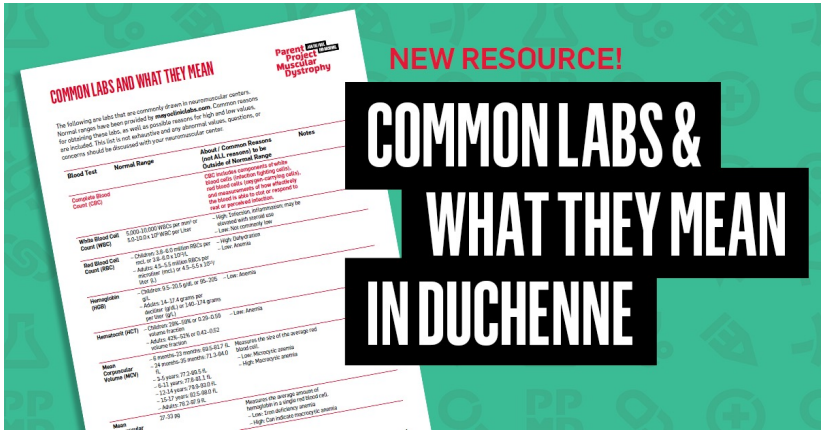
This comprehensive guide addresses the questions most often asked by carriers.

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

parentprojectmd.org/care-and-support-materials

Care & Support Materials





Resources for School

HI! MY NAME IS:

MY PHOTO

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

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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

Duchenne/Becker muscular dystophy (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.

EMERGENCY

STUDENT INFORMATION

Name: ☐M ☐F D.O.B.

Address:

C CONTACTS

Personal:

Name: Name:

Relation: Relation:

Phone: Phone:

Address: Address:

Medical:

Neuromuscular Specialist/Hospital: Primary Care Provider:

Phone: Phone:

After Hours Phone: After Hours Phone:

A ALLERGIES AND PRECAUTIONS

Allergies:

DO NOT GIVE THESE MEDICATIONS

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

R REVIEW MEDICATIONS AND MEDICAL CONDITIONS

Medications:

- ☐ **Duchenne Muscular Dystrophy**
- ☐ Obstructive Sleep Apnea
- ☐ Cardiomyopathy
- ☐ Adrenal Insufficiency
- ☐ Asthma
- ☐ Diabetes
- ☐ Autism
- ☐ Cognitive Delay
- ☐ Pacemaker/ICD
- ☐ Visually Impaired
- ☐ Hearing Impaired
- ☐ High Blood Pressure
- ☐ Kidney Disease
- ☐ Malignant Hypothermia
- ☐ Seizure Disorder
- ☐ Other:

E EMERGENCY CARE INFORMATION

Scan the QR code or visit
parentprojectmd.org/emergencycare

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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).**

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 moderate/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/DOCTOR:

NEUROMUSCULAR CENTER EMERGENCY NUMBER:

FOR FAMILIES

DUCHENNE MUSCULAR DYSTROPHY EMERGENCY CARE INFORMATION FOR HEALTHCARE PROVIDERS

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, inhaled 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.
- AST/ALT are normally elevated in patients with Duchenne and need no further evaluation.

Parent Project Muscular Dystrophy

FOR HEALTHCARE PROVIDERS

Parent Project Muscular Dystrophy
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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 PPMD Steroid Care Page at parentprojectmd.org/steroids.

Background/Assessment

Normal basal secretion of cortisol from the adrenal gland is approximately 5-7 mg/m2/day 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 perioperative 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):

PPMD for YOU

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 Moeschen, 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