# CARE CONSIDERATIONS CHECKLIST
# EARLY AMBULATORY STAGE/CHILDHOOD

Parent Project Muscular Dystrophy
JOIN THE FIGHT. END DUCHENNE.

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, like a waddling type of walk, 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 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 right 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 tests at each visit. This evaluation occurs 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 or has difficulty walking, also known as the plateau phase.
- ☐ Never stop 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 image). These show your child's heart rate, rhythm, structure, and function.
- ☐ Start taking heart medications when cardiac tests show a decline in heart function or scarring (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.

- ☐ Your child should have regular pulmonary function testing (PFT) starting at the time of diagnosis or by age 6. 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 injectable flu vaccine every year and pneumococcal vaccine as recommended.
- ☐ Your child's pulmonologist may suggest a cough assist if your child's cough is weakening.
- ☐ Your child's pulmonologist may suggest a sleep study if there are signs and symptoms of shallow breathing, called hypoventilation, while sleeping.

## NOTES

### Rehabilitation Care

The rehabilitation team may include a physiatrist, physical therapist, occupational therapist, or speech-language pathologist. The NMS will work closely with your child's rehabilitation team on your child's physical assessments.

- ☐ See a physical therapist every 4-6 months for evaluation.
- ☐ Get guidance on daily stretching and use of braces or corrective devices (i.e. AFOs or "moon boots").
- ☐ Discuss safe and appropriate daily activities, such as swimming or cycling.
- ☐ Minimize the fall risk in your child's environments, including the home, school, transportation vehicles, etc.
- ☐ See a speech therapist for speech and language concerns.

## ORTHOPEDIC AND SURGICAL CARE

An orthopedic surgeon is a doctor who specializes in the bones, muscles, joints, tendons, and ligaments.

- ☐ Provider monitors for curvature of the spine, known as scoliosis, every year by X-ray or visual inspection.
- ☐ If your child begins to have ankle contractures (shortening of Achilles tendon) consider surgery or serial casting to maintain ambulation.
- ☐ Talk to your child's cardiologist and pulmonologist when considering any surgery.
- ☐ Talk with the anesthesiologist about safe and unsafe anesthesia for your child before surgery. A list may be found at **ParentProjectMD.org/Surgery.**

## ENDOCRINOLOGY AND BONE HEALTH CARE

An endocrinologist is a doctor who specializes in hormones, the chemicals in the body that influence puberty, growth, and bone health.

- ☐ Have blood tests to measure vitamin D and calcium levels before starting steroids annually thereafter. Take vitamin D and calcium supplements as needed.
- ☐ Imaging tests to monitor the health of your child's spine and bones are recommended. The provider may order an X-ray of the spine from the side (a lateral X-ray) every 1-2 years and a DEXA scan (dual-energy X-ray absorptiometry), which measures bone density, every 2-3 years.
- ☐ See a bone health expert at the earliest sign of fracture or back pain.
- ☐ Provider measures standing height and weight every 6 months.
- ☐ Provider measures non-standing growth by measuring arm or leg length every 6 months.
- ☐ Provider monitors pubertal development every 6 months, starting at age 9.

## GASTROINTESTINAL AND NUTRITIONAL CARE

A gastroenterologist is a doctor who specializes in the digestive system. A registered dietitian nutritionist (RDN) is an expert in food and nutrition. Your child may be referred to these types of providers to address issues with weight gain or loss, nutrition, and digestion.

- ☐ See a Registered Dietitian Nutritionist (RDN) at every clinic visit if appropriate.
- ☐ Review your child's fluid and diet history and develop a nutrition plan with the RDN if needed.
- ☐ Monitor weight and BMI at each visit.
- ☐ Have an assessment for problems with swallowing, acid reflux, digestion, and constipation at every visit. You may be referred to a gastroenterologist if needed.
- ☐ See a dentist every 6 months

## NOTES

## PSYCHOSOCIAL CARE

Psychosocial health (mental health and social well-being) is as important as physical health. Your child's multidisciplinary team should include a psychologist, social worker, and/or psychiatric nurse/advanced practice nurse.

- ☐ Discuss your child's and your family's mental health at each visit. Formal or informal mental health screenings can be completed by a provider.
- ☐ See a psychologist to complete a comprehensive assessment of your child's development, learning, and behavior within the first year of diagnosis. This is referred to as a neuropsychological evaluation. Having a baseline assessment early will help your child moving forward; it will be repeated as needed.
- ☐ Before starting school, discuss the need for special school plans or accommodations. These can include an individual education plan or a 504 plan which can be developed with the help of your providers and school representatives.

## TRANSITIONS OF CARE

Planning for the future and teaching children to manage their own medical care is an important part of helping them become independent adults with Duchenne. This phase is referred to as transitioning.

- ☐ Promote age-appropriate independence and responsibilities.
- ☐ Discuss plans for the future with your child.
- ☐ Talk to your child's provider on getting your child ready to participate in healthcare decisions.
- ☐ Keep your child engaged and active with family, friends, and peers.

## EMERGENCY CARE

Emergency room staff may not have experience caring for people with Duchenne, so it is helpful to bring emergency information with you that is specific to Duchenne. This includes information on anesthesia, fracture management, oxygen, steroid stress dosing, and more. PPMD has this information available on their website (**ParentProjectMD.org/Emergency**) to print or it is available within their free Apple or Android App (**ParentProjectMD.org/App**).

- ☐ Have emergency information, either on a card or a mobile app, with you and with your child at all times.
- ☐ Have a summary of your child's last NMS visit, provided by your NMS, with you and with your child at all times. This will include the diagnosis, all medications, recent test results (including heart and lung function), and any reoccurring medical problems your child may have.
- ☐ Take any equipment that your child uses and their medication with you to the emergency room or hospital.
- ☐ Contact your NMS if your child needs to go to the emergency room or hospital for any reason.
- ☐ If your child is taking steroids, have a copy of the PJ Nicholoff Steroid Protocol (available at **ParentProjectMD.org/PJ**) which provides information on preventing adrenal insufficiency and indications for stress dosing of steroids.

## NOTES

## PRIMARY CARE

Primary care providers (PCPs) are doctors, nurse practitioners, or physician assistants who specialize in pediatrics, family, or internal medicine. This provider should work closely with your child's NMS.

- [ ] Continue seeing your child's primary care provider for routine visits, including well checks, minor illnesses, and immunizations.

## INFORMATION AND SUPPORT

Duchenne muscular dystrophy can be devastating and difficult for families. There is a lot to learn about this disease, and you may feel overwhelmed. Families who have been through this process suggest learning at your own pace, with resources and support from the PPMD community.

– Parent Project Muscular Dystrophy (PPMD): **ParentProjectMD.org**

***Genetic Testing of Family Members***

*Because Duchenne can run in families, your NMS or a genetic counselor should talk to you about which family members are at risk and offer testing. Young males in your family should receive testing for Duchenne, even if they have no signs or symptoms. The females in your family may be at risk for carrying the gene change that causes Duchenne. Females who are carriers of Duchenne have an increased risk of heart muscle disease, known as cardiomyopathy. They should be referred to a cardiologist and have a checkup every 3-5 years, even if there are no signs of cardiomyopathy.*

## NOTES

The information in these checklists are based on the 2018 care considerations for Duchenne muscular dystrophy. This set of 3 articles was published in the Lancet Neurology and funded by the Centers for Disease Control and Prevention. Learn more here: **https://www.cdc.gov/ncbddd/musculardystrophy/care-considerations.html**