CERTIFIED DUCHENNE CARE CENTER PROGRAM

Standards of Certification
Version 5.0, Approved February 2025

Parent Project Muscular Dystrophy

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STANDARDS FOR CERTIFICATION

SUMMARY

Parent Project Muscular Dystrophy (PPMD) has been a strong advocate for the provision of Duchenne-specific, comprehensive, standardized care and management for people living with Duchenne muscular dystrophy, and publicly communicating to the community the locations where that level of care & research is being provided. The Certified Duchenne Care Center Program (CDCC Program), a unique program of PPMD, aims to ensure all individuals with Duchenne have access to high quality, comprehensive and coordinated care and research. The network aims to standardize and improve care through the evaluation and certification of centers that provide comprehensive clinical care and services and research centers that focus on improving outcomes for people living with Duchenne muscular dystrophy. Centers that meet the CDCC Program criteria, and follow processes described in the standards set forth in this document, may be certified as a Certified Duchenne Care Center (CDCC) with the option of an additional Clinical Research Designation. Information regarding Certified Duchenne Care Centers, including clinical services available, number of clinical trials, and additional pertinent center information will be made publicly available on PPMD's website. The program is led by PPMD's Care Team and advised by the CDCC Certification committee. Program development and updates will be guided by family members, individuals, and US healthcare professionals through an independent Family Advisory Board and a Certification Committee. The certification process will consist of completion of the Certified Duchenne Care Center application and/or the Clinical Research Designation application, Clinical and Subspecialty Services Document, a virtual pre-site visit, an in-person site visit including faculty and staff interviews, as well as a review of patient records. People living with Duchenne muscular dystrophy, their families, and the greater Duchenne community can be confident that CDCCs are providing standardized, comprehensive care that is in alignment with the care standards endorsed by the CDCC Program. Identifying Certified Duchenne CareCenters and those with Clinical Research Designation across the United States will aid in improving patient access to comprehensive Duchenne care & clinical trials. The "Certified Duchenne Care Center Program: Standards for Certification," is a living document; updates to the program and process will be made as appropriate.

OVERVIEW OF PROGRAM

Mission of the Certified Duchenne Care Center Program

To identify/certify centers across the United States who are providing comprehensive care and clinical services to people living with Duchenne, and to communicate that knowledge to the Duchenne community, including patients, families, caregivers, national organizations, private and publicly-held companies, government institutions, industry and other key stakeholders.

Vision Statement & Guiding Principles

Our vision is that all individuals with Duchenne muscular dystrophy and their families have access to high quality, comprehensive and coordinated care and research participation. We do this through 5 guiding principles**:**

1. **Quality -** *PPMD strives to ensure that all individuals & families have access to high quality care & clinical research*

2. **Advancement -** *PPMD drives continuous improvement in the care & research provided to individuals and families living with Duchenne*

3. **Education -** *PPMD ensures that families, individuals, and health care providers have the most updated information necessary to provide and receive cutting edge care, research, support and resources*

4. **Collaboration -** *PPMD unites families and clinicians to drive connection, community, and teamwork to advance the best care and clinical research possible*

5. **Transparency -** *PPMD provides families and individuals with the information needed to make informed decisions related to care & research opportunities*

Goals of the Certified Duchenne Care Center Program

The Certified Duchenne Care Center Program will:

- Utilize the CDCC Standards to evaluate care and clinical services provided to people living with Duchenne and cared for by centers applying for certification
- Optimize and standardize care and clinical services for people living with Duchenne muscular dystrophy who are cared for by Certified Duchenne Care Centers
- Certify centers who are providing comprehensive care and clinical services to patients living with Duchenne across the US
- Publicly communicate to the Duchenne community which centers are certified as Duchenne Care Centers and facilitate connections to relevant care teams
- Share information collected from certified centers with the community to demonstrate the care and services provided at each center
- Provide guidance to those centers who are engaged and committed, but do not yet meet the criteria for certification, to reach the level of optimal care necessary for certification

Benefits of Certification

Certified Duchenne Care Centers will have access to other certified centers within this network, with the ability to discuss and address issues related to improving and enhancing care within the network. Centers will also have access to educational and supportive materials to help navigate patient care. CDCCs will have the opportunity to attend conferences, educational webinars and other beneficial opportunities provided by PPMD. CDCCs are recognized as the gold standard of Duchenne care and serve as a referral source for families seeking optimized, comprehensive care. Grant, award, and publication opportunities may arise to members of the CDCC network. Certification, once granted, will be valid for 5 years, provided requirements for certification continue to be met. Centers may apply for recertification after 5 years.

DEVELOPMENT OF PROGRAM

Certification Committee

The CDCC Program Certification Committee is composed of sub-specialists who are recognized by the Duchenne community as experts in the provision of Duchenne care and are selected and directed by PPMD's CDCC Program Director. The CDCC Certification Committee works with the Family Advisory Board for guidance of the program. Committee members agree to serve for a 3-year term, and may serve subsequent terms.

Responsibilities of the CDCC Program Certification Committee include:

- Review and approve the Certified Duchenne Care Program process and draft documents
- Assure that the CDCC Program and documents are in alignment with published standards of care.
- Review of all applications submitted by centers applying for care certification or clinical research designation
- Participation in calls with applying centers, as needed, to discuss the application and certification process
- Participate in virtual and in-person site visits of applicant centers (at least 1 member of the Certification Committee and 1 PPMD staff member will attend each site visit), which includes interviews of faculty and staff, and patient records review.
- Each member of the Certification Committee is expected to participate in up to 2 in-person certification site visits per term, but may participate in additional visits if they wish. All travel costs will be reimbursed.
- Provide recommendations and guidance that will enhance the care and services provided by CDCCs, and assist centers who do not yet meet the criteria for certification to reach the level of care necessary for certification
- Participate in regular meetings reviewing progress or to address concerns

The goals of the CDCC Program Certification Committee are:

- To certify centers across the United States who are providing comprehensive care & clinical research opportunities to patients living with Duchenne
- To ensure consistent and fair evaluation of applications
- To participate in the continued monitoring of certified centers
- To provide assistance and guidance to centers who wish to become Certified Duchenne Care Centers

Family Advisory Board

The patient voice is a critical aspect of the care ecosystem and, as such, PPMD aims to incorporate the voice of the community into the CDCC program via two mechanisms: the Family Advisory Board and the patient experience survey. The Family Advisory Board is an invitation-only committee of 10-15 family members, caregivers and individuals with Duchenne or Becker to provide insights about their experiences about access to care and how care is provided across the country. Furthermore, individuals on the board will identify educational needs related to Duchenne care and how access to care can be supported. The board will meet quarterly and individuals will serve for three year terms.

The members of the family advisory board will not participate in site visits or decisions around the certification of centers, which is the role of the CDCC Program Certification Committee. The CDCC Family Advisory Board will operate under the auspices of PPMD's CDCC leadership.

Network Designations

A center may receive designation as a Certified Duchenne Care Center. They may also elect to pursue a clinical research designation.

Certified Duchenne Care Center (CDCC)

A Certified Duchenne Care Center demonstrates commitment to excellence in providing Duchenne-specific care and management in a comprehensive, standardized manner. Certification is voluntary and demonstrates the center's commitment and accountability to deliver comprehensive care and management to people and families living with Duchenne. Certification of Duchenne Care Centers will improve the quality of patient care by standardizing care and, therefore, reducing discrepancies in care across the United States.

Clinical Research Designation

A center with Clinical Research Designation demonstrates a commitment to excellence in conducting Duchenne clinical trials and research in addition to providing comprehensive and coordinated care. Certification is voluntary and demonstrates the center's commitment and accountability to provide opportunities for clinical trials and research. These centers must be a CDCC or currently pursuing certification and this can be completed in parallel. These centers that have sufficient clinical trial infrastructure, are currently conducting multiple interventional clinical trials and have a strong history of clinical research. Centers with Clinical Research Designation will demonstrate excellence in clinical research to the community, highlighting the additional knowledge and expertise within a center distinct from provisions of clinical care.

CERTIFIED DUCHENNE CARE CENTER (CDCC)

Development of Standards

The Certified Duchenne Care Center Program is the natural evolution of PPMD's Transforming Duchenne Care Initiative (TDCI), an effort to minimize differences in the care and management of people living with Duchenne. In 2013, core members of the TDCI met to discuss the need to develop a tool to evaluate the implementation of the recommendations of the DMD Care Considerations Working Group (DMDWG), which published the CDC (Centers for Disease Control and Prevention) Care Considerations, standards of Duchenne care which were made available in 2009, published in 2010, and updated in 2018 ([1],[2],[3]). Members of this Core Group included experts in sub-specialty Duchenne care from across the United States. The result of this effort was the development of the TDCI metrics by which to evaluate care. These metrics were developed in agreement with the CDC Care Considerations[1].

The DMDWG publications identified 8 domains necessary for comprehensive care. These domains included:

- Diagnosis
- Neuromuscular management
- Orthopedic management
- Rehabilitation management
- Pulmonary management
- Cardiac management
- GI, speech/swallowing, nutrition management
- Psychosocial management

The TDCI combined several areas from the DMDWG publication, and added one new domain (communication and coordination), identifying 5 total domains deemed necessary for comprehensive care:

- Communication and coordination, an area not addressed by the care considerations, but deemed important
- Neuromuscular care (which includes neurology, physical medicine and rehabilitation and, physical therapy care, neurodevelopmental/psychosocial care)
- Cardiology Care
- Pulmonary Care
- Endocrinology Care and Bone Health
- Transition care

The members of each sub-specialty domain developed measures by which to evaluate care within their sub-specialty. The 5 domains and their measures are included in the Certified Duchenne Care Center Application. To be eligible for certification, centers will need to complete the Certified Duchenne Care Center Application, demonstrating that clinical practice is consistent with these measures. The information gathered

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[1] Birnkrant, D. J., Bushby, K., Bann, C. M., Apkon, S. D., Blackwell, A., Brumbaugh, D., Case, L. E., Clemens, P. R., Hadjiyannakis, S., Pandya, S., Street, N., Tomezsko, J., Wagner, K. R., Ward, L. M., & Weber, D. R. (2018). Diagnosis and management of Duchenne muscular dystrophy, part 1: Diagnosis, and neuromuscular, rehabilitation, endocrine, and gastrointestinal and nutritional management. *The Lancet Neurology*, *17*(3), 251–267. https://doi.org/10.1016/s1474-4422(18)30024-3

[2] Birnkrant, D. J., Bushby, K., Bann, C. M., Alman, B. A., Apkon, S. D., Blackwell, A., Case, L. E., Cripe, L., Hadjiyannakis, S., Olson, A. K., Sheehan, D. W., Bolen, J., Weber, D. R., & Ward, L. M. (2018). Diagnosis and management of Duchenne muscular dystrophy, part 2: Respiratory, cardiac, bone health, and Orthopaedic Management. *The Lancet Neurology*, *17*(4), 347–361. https://doi.org/10.1016/s1474-4422(18)30025-5

[3] Birnkrant, D. J., Bushby, K., Bann, C. M., Apkon, S. D., Blackwell, A., Colvin, M. K., Cripe, L., Herron, A. R., Kennedy, A., Kinnett, K., Naprawa, J., Noritz, G., Poysky, J., Street, N., Trout, C. J., Weber, D. R., & Ward, L. M. (2018). Diagnosis and management of Duchenne Muscular Dystrophy, part 3: Primary care, emergency management, Psychosocial Care, and transitions of care across the lifespan. *The Lancet Neurology*, *17*(5), 445–455. https://doi.org/10.1016/s1474-4422(18)30026-7

in the Certified Duchenne Care Center Application will be substantiated during pre-site visit virtual meetings, the site visit through faculty and staff interviews, as well as the review of selected patient records. Parents and patients from that center will be encouraged to participate in The Duchenne Registry, PPMD's patient reported registry. Parents and patients will also be encouraged to participate in the Patient Experience Survey, which will be used to support provider responses in the Certified Duchenne Care Center Application. Requirements for Certification, as well as information gathered from the Clinical and Sub-Specialty Services Document, will be made publicly available via the PPMD website.

As patient outcomes are collected, and in the face of new scientific evidence, standards of care will change. In this event, the variables within each required domain of care will be updated to comply with newly developed care guidelines.

CDCC Program Requirements for Care & Services

Clinical and Sub-Specialty Services

Certified Duchenne Care Centers employ teams of sub-specialists offering comprehensive sub-specialty care and clinical services to people and families living with Duchenne and Becker muscular dystrophy. Each sub-specialist will be expected to provide care according to the Certification Program Standards.

Centers must include access to the following providers (at the same facility, *during the same multi-day or single-day visit*). All providers should have expertise in Duchenne and Becker muscular dystrophy and collaborate with the clinic team.

- Clinic coordinator, preferably by a nurse and/or nurse practitioner
- Neuromuscular specialist (Neurologist and/or physical medicine and rehabilitation)
- Cardiology
- Pulmonary
- Physical Therapy (PT)
- Social work (SW)

The following sub-specialty and clinical services must have a dedicated subspecialist available as needed to see patients (at the same facility, *during or in collaboration with the same multi-day or single-day visit*) when appropriate:

- Genetic Counseling
- Nutrition
- Orthopedics
- Endocrinology
- Palliative Care
- Durable Medical Equipment (Mobility/wheelchair services)

The following sub-specialty and clinical services must be available as needed by referral to see patients (at the same or different facility, during or separate from the same multi-day or single-day visit):

- Physical Medicine and Rehabilitation (if not the primary neuromuscular specialist/provider)
- Developmental pediatrics
- GI
- Orthotics
- Occupational Therapy (OT)
- Psychology and/or/Neuropsychology
- Speech Therapy (ST)
- Surgery

Provision of Care

Care will be provided according to the 2018 standards as identified by the Duchenne muscular dystrophy, Care Consideration Working Group, and specified by the Certified Duchenne Care Center Program. Provisions of care will be applicable to both pediatric and adult centers.

Coordination and Communication

- A summary with recommendations for care is sent to the patient's PCP at the time of diagnosis and after each appointment
- Disease specific information/resources should be available and sent to PCP at time of diagnosis
- Patients/families receive a copy of the note/summary sent to their primary care provider and/or will have access to an electronic copy of their note/summary
- Patients and families have a dedicated care coordinator(s) as the point of contact to provide information & facilitate communication and coordination among clinicians across multiple disciplines
- Center has dedicated staff member to facilitate and manage access for Duchenne specific therapies
- Patients and families are provided with after hours/on call phone number and instructions on what to do in case of emergencies at every visit

Neuromuscular Specialist (Neurology/PM&R)

- Patients see the neuromuscular specialist every 6 months
- Patients are measured for height/length by standing (when appropriate) or ulnar length
- Patients are weighed every 6 months or more often as needed
- Growth curve and BMI is assessed
- The use of corticosteroids, and their side effects are discussed with every patient
- Corticosteroids are recommended during the toddler stage, before significant decline
- Corticosteroids are continued lifelong, baring side effects necessitating discontinuation and taking into account patient preferences
- Steroid complications (obesity, cushingoid features, acne, loss of bone density, behavioral changes, immune suppression, hypertension, short stature, delayed puberty, GERD/peptic ulcer disease, hirsutism, glucose intolerance, myoglobinuria) are assessed and managed at each visit
- Information on steroid conversion, stress dosing and prevention of adrenal crisis (PJ Nicholoff Steroid Protocol or equivalent institutional protocol) is provided to each patient
- Steroids use and adrenal insufficiency risk is clearly documented in EMR (through flag/alert or other avenue for cross department communication)
- FDA approved Duchenne medications are discussed and offered to each patient for whom they are appropriate
- Childhood immunizations (including pneumovax, influenza and COVID-19) are encouraged to be up-to-date and given as recommended (in US, by the CDC)
- Each patient is offered and strongly encouraged to have genetic testing at time of diagnosis

- Genetic counseling is available at each clinic, and includes discussion of genetic risk, carrier testing and preimplantation genetics (as appropriate)

Rehabilitation Management

- Patients see PT and/or PM&R every 6 months for an evaluation
- Standardized strength and functional measures are obtained every 6 months to track disease progression and therapeutic response
- Range of motion in the upper and lower extremities is assessed every 6 months
- Stretching is recommended
- Ankle foot orthoses (AFOs) are encouraged (nighttime while ambulatory and day or night with loss of ambulation) from diagnosis and/or at a very young age, prior to heel cord tightness
- Hand splints are prescribed if appropriate
- Safety/fracture prevention/complications are assessed and discussed at each visit
- If applicable, the rehabilitation team (PT/OT/PM&R) provides activity and exercise recommendations at each visit
- The need for assistive devices and adaptive equipment is assessed at each visit

Endocrinology, Orthopedics, and Bone Health

- Patients are referred to endocrine/bone health when starting corticosteroids, delayed onset of puberty, evidence of vertebral compression fracture (VCF) or first long bone fracture
- Patients are referred for endocrine evaluation if there is a downward trajectory in height percentile or <4cm growth per year, prior to full sexual maturity
- Stage of sexual maturity is evaluated at each visit after age 10 years
- Testosterone supplementation is discussed if the individual is pre-pubertal by age 14 years or has documented testosterone deficiency at older age
- The back is visually inspected for scoliosis at each visit
- Radiographic evaluations of ambulatory patients are done when there are clinical signs of scoliosis
- Scoliosis evaluation is performed annually when the curve is <15-20 degrees and every 6 months when the curve(s) >20 degrees during skeletal growth, and after growth if clinically indicated
- Patients taking steroids get a lateral lumbar spine x-ray at the initiation of steroids and every 1-2 years thereafter (every 2-3 years if not taking steroids)
- IV Bisphosphonates are discussed and offered for the management of bone fragility, with evidence of vertebral compression fracture (VCF) or with low trauma fracture
- There is an experienced orthopedic spine surgeon managing scoliosis and posterior spine fusions associated with this center
- If appropriate, patients are referred to orthopedic surgery for evaluation of heel cord surgery

Pulmonary

- Patients see pulmonologist at least annually from diagnosis
- %O2 Sat measured at least annually in non-ambulatory patients
- CO2 is measured in non-ambulatory patients

- Ambulatory patients, for whom it is appropriate, receive standard spirometry (including FVC) at least annually
- Non-ambulatory patients receive some pulmonary function assessment at least every 6 months, and more often as needed
- Lung volume recruitment (using self-inflating manual ventilation or mechanical insufflation-exsufflation device) is discussed/recommended to be used 1-2 times/day when FVC <60% predicted
- Cough peak flow is measured at least annually and more often as needed
- Assisted cough is offered when cough peak flow is below 270 LPM, FVC <50% or MEP <60 cm H2O
- Patients assessed for symptoms of sleep related hypoventilation (nighttime awakenings, morning headaches, behavioral changes) at each visit
- Sleep studies offered for signs/symptoms of obstructive sleep apnea or sleep disordered breathing
- Sleep studies discussed/offered when FVC is <50% predicted
- Nocturnal NIV (with backup rate of breathing) is offered to patients with FVC <50%, MIP <60 cm H2O or awake baseline SpO2 <95% and or with evidence of sleep related hypoventilation on polysomnogram.
- Daytime NIV is discussed with patients whose daytime exhaled CO2 >45 mmHg or greater, SpO2 <95% or with symptoms of awake dyspnea are present

Cardiology

- All patients should see cardiology at the time of diagnosis for baseline evaluation to include ECG and non-invasive cardiac imaging
- All individuals should be evaluated by cardiology at least annually or more frequently as necessitated by clinical course
- Patients have an ECG done at least annually
- Individuals should have electronic access to or a printed copy of their ECG to use in emergencies
- Every patient receives cardiac imaging at least annually. Cardiac MRI (CMRI) is the imaging modality of choice for children over the age of 7-8 years if it can be done without sedation/anesthesia. Echocardiography can be performed in settings where CMRI cannot be performed or if the patient cannot tolerate CMRI.
- ACEi or ARB should be started by age 10 years or with evidence of cardiac dysfunction or fibrosis
- Patients taking ACEi or ARB have their BUN, creatinine and electrolytes checked annually
- Maternal carriers should be identified and at-risk individuals referred for appropriate cardiac screening

Nutrition

- A registered or licensed dietitian (RD) is part of the team
- The RD is available to see patients at the start of steroids and at each visit, as appropriate, thereafter

- The RD is able to help each family develop a nutrition plan, helping to prevent obesity or treat underweight
- Vitamin D 25 OH is monitored just prior to starting steroids and repeated annually
- Intake of calcium is assessed at each visit and supplements encouraged as needed
- Swallowing, constipation, GERD and gastroparesis is assessed at each visit

Palliative Care

- Palliative care is discussed/offered/encouraged for patients and their families at time of diagnosis and throughout the lifespan
- Palliative care is available as part of the team or by referral

PsychosocialNeurodevelopmental

- Developmental evaluation including speech and language, assessment of autism spectrum disorder, and referral to speech therapy, developmental medicine, and neuropsychology as needed
- Evaluation of emotional adjustment/coping, emotional disorders and social isolation is done at each visit and referral to behavioral health specialist as needed
- The mental health of the individual, caregiver(s) and family is assessed at each visit with referrals as appropriate
- Social determinants of health for patient and family are assessed at each visit
- Ongoing support is provided either at your center or referrals made to outside providers

Transition to Adulthood

- Patients >14 years old spend at least a portion of their visit alone (without caregiver present) with a clinic team member at each visit
- Assessment and discussion of transition process occurs at least annually at age 14 and older
- Transition plan and discussions include:
    - Consistent person to monitor the transition plan
    - Discuss & set age appropriate future goals
    - Discuss social and community participation and friendships/relationships
    - Plans for adult healthcare, education/employment, adult living, insurance considerations
    - Healthcare power of attorney (POA), guardianship or other supports that may be helpful by 18 years of age
    - Advanced care planning/advance directives/emergency care plan in place by 18 years of age

CDCC Certification Process & Documents

New applications for certification will be accepted on a rolling basis. PPMD CDCC program leadership, under guidance of the CDCC Certification Committee, retains the right to prioritize program evaluation based on patient need, organizational priorities, and staffing bandwidth, which may include limiting the number of new certifications or site visits completed annually.

Criteria for Centers Applying for CDCC Certification

Centers will be considered eligible to apply for certification if it meets the following criteria:

- Provides care according to the Duchenne Care Considerations, which is demonstrated by the CDCC application, substantiated by the site visit and supported by patient reported outcomes in the Duchenne Registry and parent/patient responses in the Patient Experiences survey.
- Acts in good faith in providing complete and accurate information during the certification process and during its term of certification.
- Agree to encourage/enable patients/families to annually update their profile in The Duchenne Registry and complete the Patient Experiences Survey

CDCC Certification Process

The process to obtain certification as a Certified Duchenne Care Center is as follows:

- Requests for certification applications will be made by the requesting center, at which time the CDCC Program Director will engage with the clinic to assess site qualifications and provide feedback on necessary steps prior to submitting an application.
- Standards for Certification document and application will be sent to the requesting applicant. Once the application is submitted in full, a preliminary assessment will be done by the PPMD care team and/or CDCC Program Director. Supplemental information may be required upon request.
- Application will be reviewed by the Certification Committee. If accepted by the committee to move forward, in-person and virtual pre-site visits will be scheduled.
- Virtual pre-site visit will include review of application and overview of in person site visit, and patient chart reviews.
- In person site visits will include attendance at multidisciplinary clinic, staff and faculty interviews, review of patient records, and family interviews as appropriate.
- A post site visit huddle will take place within one week of completion of the in-person visit to discuss identified requirements for certification or other suggestions that may be helpful to the team as well as discussion of potential solutions and/or plans moving forward.
- Application packet including site visit summary and post huddle notes will be sent to certification committee for review and vote
- If any requirements are identified, clinic leadership is informed and the clinic will be given 6 months to implement care updates, at which point a virtual check in will be scheduled. If the clinic is unable to meet requirements within 1 year, they must reapply.
- If all requirements are met and the certification committee votes for approval, certification is granted.
- Once certified, the CDCC Program Director will send necessary documents to the newly certified clinic including notice of award, certification agreement & joint press release
    - Newly certified clinics will agree to announce certification jointly with PPMD
- Certified Clinics will:
    - Comply with the decisions and recommendations of the Duchenne Care Center Certification Committee, which will be evaluated annually
    - Agee to encourage and enable patients and families to register, and annually update, information in both The Duchenne Registry and the Patient Experiences Surveys.

CDCC Site Visits

Virtual Pre-Site Visit

Prior to the in person site visit, at least 1 member of the Certification Committee and 1 PPMD care team member will meet virtually with the clinic director(s) and coordinator(s) to review the application and in person site visit process. Patient charts may be reviewed as part of the virtual pre-site visit. If major staffing or program structure changes are identified during the virtual site visit, the in person site visit may be postponed.

In Person Site Visit

Site visits will be attended by at least 1 member of the Certification Committee and 1 PPMD care team member. The visit tasks of the certification committee members will include:

- Substantiation of the clinical and subspecialty services reported in application
- Substantiation of care reported on the CDCC Application
- 4-6 patient charts will be reviewed using an Excel checklist (no PHI will be collected)
- Faculty will be interviewed regarding services and care provided
- Staff will be interviewed regarding services and care provided
- Clinic tour to evaluate the flow and services provided to patients and families
- Evaluation of patient/family/provider flow during a normal comprehensive neuromuscular visit

Centers Unable to Meet CDCC Certification Criteria

Centers who are engaged and committed to the provision of comprehensive Duchenne care, but who are not yet able to meet the full criteria for certification will be given recommendations and guidance from the CDCC Certification Committee. Due to the high demand of CDCC requests if a center requires more than minor adjustments we encourage them to apply the following calendar year.

Should a certified center have a change in services available that substantially impacts their qualification for certification, revocation of certification may occur after a review by the certification committee.

CDCC Annual Review

Certification will be granted for 5 years, providing that centers:

- Update (at least annually) both the Clinical and Subspecialty Services Document and pertinent clinical details as needed
- Continue to provide care and services in alignment with the CDCC Program.
- Continue to encourage/enable their parents/patients to maintain and update their Patient Profile in The Duchenne Registry and complete the Patient Experience Survey annually.
- Demonstrate continued quality improvement and compliance with recommendations made by the Certification Committee, which is reviewed annually
- Maintain clear and open channels of communication with PPMD and the CDCC Certification Committee
- Provide annual report documentation and complete annual meeting with PPMD CDCC staff

At the end of a 5-year cycle, centers will again be eligible for recertification.

Certified Duchenne Care Center (CDCC) Recertification

Centers may apply for recertification every 5 years. To be eligible for recertification, centers must complete the following:

- Complete the online Certified Duchenne Care Center application at time of initial certification and recertification. This includes updating the Clinical and Subspecialty Services Document
- Need for a recertification site visit will be determined by PPMD staff for recertification

- For both in person and virtual recertification, a virtual chart review for CDCCs will be scheduled and completed by PPMD staff and certification committee member if necessary
- For an in person site visit, at least 1 PPMD staff member will shadow in the clinic and conduct staff interviews. If there are major changes or concerns it would be suggested to bring a committee member.
- The Certification CDCC Committee will review application materials, summary of site visit and chart review, and vote to recertify
- Centers must comply with the decisions and recommendations of the Duchenne Care Center Certification Committee, which will be evaluated annually

Certified Duchenne Care Center Application

This will be completed and submitted online via WebGrants

Center information

Name of institution/clinic/center:

Address of institution/clinic/center:

Phone number of clinic/center:

Main Hospital/ institution/ center website:

Clinic/Center director's name (if there is more than 1 director, please list the one most involved):
Name:
Title:
Email:

Address:
Phone:
Fax:

Specialty/subspecialty board certification of the director listed above (choose all that apply):
☐ Pediatrics
☐ Adult, Internal or
☐ Family medicine
☐ Adult neurology
☐ Child neurology
☐ Adult PM&R
☐ Other: _____

Additional certifications (choose all that apply):
☐ Neuromuscular medicine (Board certified in Neurology, Child Neurology or PM&R)
☐ Pediatric PM&R (Board certified in PM&R)
☐ Child Neurophysiology (Board certified in Neurology, Child Neurology or PM&R)
☐ Other: _____

Time clinic/center director (named above) has treating pediatric patients (0 -21 years) with Duchenne muscular dystrophy
☐ 1-5 years
☐ 6-10 years
☐ >11 years

Time clinic/center director (named above) has treating adult patients (>22 years) with Duchenne muscular dystrophy
☐ 1-5 years
☐ 6-10 years
☐ >11 years

Person(s), title and number for families or providers to call for an appointment:
Name:
Title:
Number:

Person(s), title and phone number for families or providers to call with concerns/questions:
Name:
Title:
Number:

Clinic Coordinator(s):
Name:
Title:
Email:
Number:

How many patients with Duchenne and/or Becker do you currently care for?
☐ Less than 20
☐ 20-50
☐ 50-100
☐ 100-200
☐ More than 200
If over 200, approximate number of Duchenne patients seen per year: _____

Clinic Structure & Contacts

In your center, Duchenne/Becker patients are seen:
☐ In a multidisciplinary clinic- By three or more sub-specialty providers and additional services available on the same day, in the same clinic
☐ In a partial multidisciplinary clinic- By three of more sub-specialty providers and additional services on multiple days, in multiple locations
☐ By single provider(s)- separate appointments for each sub-specialists and additional services

How would you describe your center in relation to Duchenne and Becker specific clinical trials?
**Participation in clinical trials is not a requirement for certification* (select all that apply)
☐Clinical Trials available at this center
☐Research studies (non-interventional) and non-drug trials available at this center
☐Referral to outside center for clinical trials
☐No clinical trial or referrals available for patients at this center
☐Comment _____

Sub-specialists	Provider Name	Email (PPMD use only)	A.) Available in neuromuscular clinic	B.) Available same day but different location	C.) Available different day by referral	D.) Refer to outside center (please list center and physician name if known)	E.) Not available
Neurology Pediatric							
Neurology Adult							
Nurse/Clinic Coordinator(s)							
Cardiology Pediatric							
Cardiology Adult							
Pulmonary Pediatric							
Pulmonary Adult							
PM & R Pediatric							
PM & R Adult							

Please complete the attached Clinical and Sub-Specialty Services Document with providers who are available through your clinic/center to provide care to patients with DBMD:

GI/GU Pediatric							
GI/GU Adult							
Orthopedics Pediatric							
Orthopedics Adult							
Endocrine Pediatric							
Endocrine Adult							
Palliative Care Pediatric							
Palliative Care Adult							
Developmental Pediatrics							
Surgery Pediatric							
Surgery Adult							
Other:							

Services	Provider Name	Email (PPMD use only)	A.) Available in neuromuscular clinic	B.) Available same day but different location	C.) Available different day by referral	D.) Refer to outside center (please list center and physician name)	E.) Not available
DME (vendor, distributor)							
Genetic Counselor							
Nutrition							
Orthotics							
OT							
PT							
Psychology/neuropsychology/counseling							
Social Work							
Speech Therapist							
Wheelchair Specialist							

Research Coordinator(s)							
Exon Skipping Therapy/Infusion Center							
Other (please specify):							

Tests	Contact (if applicable)	Email (PPMD use only)	A.) Available in neuromuscular clinic	B.) Available same day but different location	C.) Available different day by referral	D.) Refer to outside center	E.) Not available
PFTs							
Echocardiogram							
EKG							
Cardiac MRI							
DEXA scan							
Labs							
other (please specify):							

Do you have outreach clinic locations where patients with DBMD can be seen?
☐ Yes
☐ No

If yes, please give the location and addresses of your outreach clinic locations: _____

If you have outreach clinics, which of the following services/sub-specialties are available to patients on the day and/or location of the outreach clinic appointment? (Choose all that apply):

Same Day	Referral
☐	☐ Cardiology
☐	☐ Developmental pediatrics
☐	☐ DME
☐	☐ Endocrine
☐	☐ Genetic counselor
☐	☐ GI/GU
☐	☐ Neurology
☐	☐ Orthotics
☐	☐ Other _____
☐	☐ Palliative care
☐	☐ PM&R
☐	☐ Psychology/neuropsychology/counseling
☐	☐ PT
☐	☐ Pulmonary
☐	☐ Social Work
☐	☐ Speech
☐	☐ Surgery
☐	☐ Wheelchair specialists

How long does it usually take for a patient with a new diagnosis to be seen?
☐ Less than 2 weeks
☐ Between 2 weeks and 1 month
☐ Between 1 month and 3 months
☐ 3-6 months
☐ 6-12 months
☐ More than 12 months
☐ Other: _____

At what age does your center transition patients to adult care?
_____

Coordination and Communication

1. Does your NM Clinic/Center have a dedicated care coordinator(s) and/or a point of contact to provide information to families & facilitate communication and coordination among clinicians across multiple disciplines?
☐Yes
☐No
☐Comment ____

2.

a.	Does your NM Clinic/Center provide a written summary with recommendations to the patient's PCP at the time of diagnosis?	☐No ☐Yes
b.	Does your NM Clinic/Center provide a written note with recommendations to the patient's PCP with each appointment?	☐No ☐Yes
c.	Does your NM Clinic/Center provide the patient's PCP office with written resources regarding the care of patients with Duchenne? (e.g. reference list or list of Internet resources)	☐No ☐Yes
d.	Does your NM Clinic/Center provide the family with written summary & recommendations?	☐No ☐Yes
e.	Does your NM Clinic/Center use an electronic record that allows the patient/family to access their medical records?	☐No ☐Yes

**Questions: YES indicates that this is correct for 90% of patients for whom the care is appropriate and accepted by the patient/parent*

Neuromuscular Provider (PM&R/Neurology)

Does your NMS see patients every 6 months?
☐ Yes
☐ No
☐ Comment _____

Is pain assessed at every clinic visit?
☐ Yes
☐ No
☐ Comment _____

Are patients weighed at each visit?
☐ Yes
☐ No

If yes, how are patients weighed?
☐ Standing or sitting on a scale in light clothes, no shoes, no braces
☐ Seated in a wheelchair followed by weighing of the wheelchair

☐ Seated in a wheelchair using pre-recorded wheelchair weight
☐ Hoyer lift
☐ Exam table

Is BMI and/or growth curve assessed on all patients?
☐ Yes
☐ No

Is patient height/length measured at each visit?
☐Yes
☐No
☐Comment _____

If yes, what measurement (s) of height/length are used?? (please choose all that apply)
☐ Ulnar length
☐ Standing height
☐ Arm span
☐ Other _____

Is corticosteroid treatment discussed with every patient?
☐ Yes, everyone
☐ Yes, but only with selected patients
☐ No
☐ Comment

When are corticosteroids typically started?
☐ Toddler stage (typically before age 5 years, before significant decline)
☐ At plateau, when physical function begins to decline
☐ Other: _____

Once corticosteroids are started, baring side effects necessitating discontinuation, how long is steroid therapy maintained?
☐ Lifelong
☐ Until loss of ambulation
☐ Other: _____

After corticosteroids are started, baring side effects, the dose is usually:
☐ Adjusted for height/weight as needed
☐ Maintained at the starting dose regardless of height/weight changes
☐ Other_____

Are steroid complications (Obesity, Cushingoid features, acne, loss of bone density, behavioral changes, immune suppression, hypertension, short stature, delayed puberty, GERD/peptic ulcer disease, hirsutism, glucose intolerance, myoglobinuria) routinely discussed and managed?
☐ Yes, complications are discussed during each visit
☐ Yes, complications are discussed during the visit and we have an information sheet we give to parents
☐ No, complications are not discussed
☐ Other _____

Is information on steroid conversion, stress dosing and prevention of adrenal crisis (PJ Nicholoff Steroid Protocol or equivalent institutional protocol) provided to each patient?
☐ Yes
☐ No
☐ Comment _____

Is steroid use and adrenal insufficiency risk clearly documented in EMR for cross department communication through a flag or alert?
☐Yes for all patients on corticosteroids
☐Only certain patients on corticosteroids,
☐No flag/alert documented in chart
☐Other _____

Are appropriate, FDA-approved Duchenne specific medications discussed and offered to each patient?
☐ Yes
☐ No

☐ Comment_____

Does your center administer commercially available gene therapy products for the treatment of Duchenne?
☐Yes
☐No
☐Comment _____

If your center does not administer commercially available gene therapy products for the treatment of Duchenne, do you have a center to whom you refer for the administration of approved therapies?
☐Yes
☐No

If yes, please list the center:
_____

Is the back visually inspected for scoliosis at each visit?
☐ Yes
☐ No

Does your clinic perform radiographic evaluations of ambulatory patients when there are clinical signs of scoliosis?
☐ Yes
☐ No

How often is a radiographic scoliosis evaluation performed?
☐ Annually when curves measure less than 15-20 degrees and every six months when curves measure greater than 20 degrees until growth is complete or longer, if clinically indicated
☐ Annually until growth is complete or longer, if clinically indicated
☐ Comment

Does every patient taking steroids get a lateral lumbar spine x-ray at the initiation of steroids and every 1-2 years thereafter (every 2-3 years if not taking steroids)?
☐ Yes
☐ No
☐ Comment

Are patients immunized/encouraged to receive influenza vaccine annually?
☐ Yes
☐ No
☐ Comment _____

Are childhood immunizations (including pneumovax) encouraged to be UTD and given as recommended by the CDC?
☐ Yes
☐ No
☐ Comment

Therapy Assessment

Are patients seen by PT/PM&R every 6 months for an evaluation?
☐ Yes
☐ No
☐ Comment _____

Are standardized strength measures obtained every 6 months to track disease progression and therapeutic response?
☐ Yes
☐ No
☐ Comment _____

If yes, what strength measures are being used?
☐ Manual muscle testing
☐ Hand held dynamometer

☐ Hand grip
☐ Pinch grip
☐ Other: _____

Are standardized functional measures obtained longitudinally to track disease progression and therapeutic response at least annually?
☐ Yes
☐ No
☐ Comment _____

If yes, what standardized functional measures are used?
☐ Time to stand/ Timed Gowers
☐ 10 meter walk/run
☐ 30 meter walk/run
☐ 6 min timed walk
☐ 2 min timed walk
☐ Time to climb 4 stairs
☐ North Star Ambulatory Assessment (NSAA)
☐ Performance of Upper Limb Scale (PUL)
☐ Brook Scale
☐ other (i.e., 100 meter, etc.) _____

Is range of motion (ROM) assessed every 6 months?
☐ Yes
☐ No
☐ Comment _____

Is daily stretching encouraged from diagnosis?
☐ Yes
☐ No
☐ Comment _____

Are AFOs (nighttime stretching orthoses/splints) encouraged? (nighttime while ambulatory and day or night with loss of ambulation)
☐ Yes
☐ No
☐ Comment _____

When are nighttime orthoses/splints prescribed?
☐ From diagnosis
☐ At point of ankle tightness
☐ At point of fixed contracture
☐ Other _____

Are hand splints prescribed if appropriate?
☐ Yes
☐ No
☐ Comment

Are safety/fracture prevention/complications assessed and discussed at every visit?
☐ Yes
☐ No
☐ Comment _____

Is there a rehabilitation professional (PM&R provider, OT, PT) providing activity and exercise recommendations?
☐ Yes
☐ No

Who assesses the need for assistive devices/equipment?
☐ PT
☐ OT
☐ PM&R
☐ Neuromuscular Neurology
☐ Other _____

Genetics

Is genetic diagnosis encouraged, offered, and available for patients?
☐ Yes for all patients
☐ Yes but only for selected patients
☐ No, not routinely offered
☐Comment _____

How is genetic testing counseling provided?
☐ Genetic counselor in clinic/available on same day to see patients
☐ Genetic counselor is available via The Duchenne Registry
☐ Referral to genetics clinic to be seen at a different location/on a different day within 6 months of diagnosis and as needed
☐ Counseling provided primarily/exclusively by neuromuscular provider
☐ Counseling by neuromuscular provider with genetic counseling referral in selected cases

Which of the following are routinely discussed/offered?
☐ Carrier testing
☐ Recurrence risk
☐ Preimplantation genetics

Endocrinology, Bone Health & Orthopaedics

When are patients referred to endocrinology? (check all that apply)
☐ When starting corticosteroids
☐ Evidence of VCF or long bone fracture
☐ Downward trajectory in height %ile or <4cm growth per year, prior to full sexual maturity
☐ Delayed onset of puberty
☐Other _____

Are IV bisphosphonates discussed for management of bone fragility, with evidence of VCF or with low trauma fracture?
☐ Yes
☐ No
☐ Comment_____

How is sexual maturity evaluated at each clinic visit after 10 years of age?
☐ Parent/patient report
☐ Evaluation by primary care
☐ Direct assessment by NMS
☐ Direct assessment by endocrinologist
☐ Other _____
☐ Sexual maturity is not assessed

Is testosterone initiation discussed if patients are found to be testosterone deficient or pre-pubertal by age 14 years?
☐ Yes
☐ No
☐ Comment _____

Does your clinic have access to an experienced pediatric spine surgeon who manages scoliosis and performs posterior spinal fusions?
☐ Yes
☐ No

If appropriate, are patients referred to orthopedic surgery for evaluation of heel cord surgery?
☐ Yes
☐ No
☐ Comment _____

Pulmonary
Do patients see the pulmonologist at diagnosis and at least annually or more often as needed thereafter?
☐ Yes

☐ No
☐ Other _____

Is %O2 Sat measured annually in all non-ambulatory patients?
☐ Yes
☐ No

Is CO2 measured annually in non-ambulatory patients?
☐ Yes
☐ No

Do ambulatory Duchenne patients, for whom it is appropriate, receive standard Spirometry (including FVC) at least annually?
☐ Yes
☐ No
☐ Other_____

Do non-ambulatory Duchenne patients receive some pulmonary function assessment at least every 6 months, and more often as needed?
☐ Yes
☐ No

Is lung volume recruitment (using self-inflating manual ventilation or mechanical insufflation-exsufflation devices) discussed/recommended 1-2 times/day when FVC <60% predicted?
☐ Yes
☐ No
☐ Comment_____

Is cough peak flow being measured at least annually and more often as needed?
☐ Yes
☐ No

If yes, is a cough assist offered when the cough peak flow is below 270 lpm, FVC <50%, or MEP <60cm H2O?
☐ Yes
☐ No

Are patients asked about symptoms of nighttime hypoventilation (nighttime awakenings, morning headaches, and behavioral changes) at each visit?
☐ Yes
☐ No

Are sleep studies offered for signs and symptoms of obstructive sleep apnea or sleep disordered breathing
☐ Yes
☐ No

Are sleep studies being discussed/offered when FVC is <50% predicted?
☐ Yes
☐ No

Is nocturnal NIV (with backup rate of breathing) offered to patients with FVC <50%, MIP <60cm H2O or awake baseline SpO2 <95% or pCO2 >45 mmHg and/or with evidence of sleep-related hypoventilation on polysomnogram?
☐ Yes
☐ No
☐ Comment _____

Is daytime NIV discussed with patients whose daytime exhaled CO2 is 45 mmHg or greater, SpO2 <95% or symptoms of awake dyspnea are present?
☐ Yes
☐ No

Cardiology

Do all patients see cardiology at the time of diagnosis for baseline evaluation to include ECG and non-invasive cardiac imaging & at least annually thereafter?
☐ Yes
☐ No
☐ Comment _____

Do all patients have an ECG done at least annually
☐ Yes
☐ No

Do all patients receive a printed copy of their ECG or have electronic access to use in emergencies?
☐ Yes
☐ No

Does every patient receive cardiac imaging (cMRI if available and appropriate, echo if cMRI unavailable, not appropriate or if every six month evaluations are needed) at least annually?
☐ Yes
☐ No
☐ Comment_____

Are cardiac medications (ACEi or ARB) started with evidence of cardiac dysfunction or fibrosis, or by age 10 years with normal findings?
☐ Yes
☐ No
☐ Comment_____

Are 24 hour Holter monitors ordered with concerns of abnormal cardiac rhythms?
☐ Yes
☐ No
☐ Comment_____

Are standard heart failure medications initiated at the onset of heart failure symptoms, with evidence of increased fibrosis, or deterioration of function?
☐ Yes
☐ No
☐ Comment

If ACEi/ARB are prescribed, are BUN, creatinine and electrolytes checked annually?
☐ Yes
☐ No
☐ Comment _____

Are identified maternal carriers routinely referred for cardiac screening?
☐Yes
☐No
☐Comment _____

Nutrition

Is there a registered or licensed dietitian (RD) on your team?
☐ Yes
☐ No
☐ Comment _____

Is the RD able to see patients at the start of steroids and at each visit (as appropriate) thereafter?
☐ Yes
☐ No
☐ Comment _____

Is the RD able to help each family develop a nutrition plan, helping to prevent obesity or treat underweight?

☐ Yes
☐ No
☐ Comment _____

Is 25-OH Vitamin D monitored just prior to starting corticosteroids and repeated annually?
☐ Yes
☐ No

Is intake of calcium assessed at each visit and calcium supplementation encouraged as needed?
☐ Yes
☐ No
☐ Comment _____

Is an informal/formal assessment of swallowing, constipation, GERD and gastroparesis done at each visit?
☐ Yes
☐ No
☐ Comment _____

Are referrals made to GI or other specialists for issues/concerns?
☐ Yes
☐ No
☐ Comment _____

Are gastrostomy tubes discussed for issues of weight loss, dehydration, aspiration and/or moderate to severe dysphasia?
☐ Yes
☐ No
☐ Comment _____

Palliative Care

Is Palliative Care discussed, offered, or encouraged for your patients?
☐ Yes, PC is part of our NM team
☐ Yes, we do discuss the advantages of PC
☐ Yes, we discuss the difference between PC and hospice care
☐ No, we do not offer PC
☐ Comment_____

Is Palliative Care available to patients?
☐ Yes, they are part of our NM team
☐ Yes, they are available by referral
☐ No
☐ Comment_____

At what age is palliative care offered or discussed with patients and families?
☐At time of diagnosis
☐Throughout the life as needed
☐At age 18 or adulthood
☐Other _____

Neurodevelopment and Psychosocial issues

Which of the following formal (using a tool) or informal (asking questions during the visit) assessments of the person living with Duchenne, are performed at each visit:
☐ Evaluation of emotional adjustment/coping Tool used_____
☐ Speech and language evaluation Tool used _____
☐ Signs of autism spectrum disorder Tool used _____
((language delays, repetitive behaviors, deficits in social functioning))
☐ Signs of behavioral disorder (ADD/ADHD, autism, OCD) Tool used_____
☐ Signs of emotional disorder (anxiety, depression) Tool used _____
☐ Signs of social isolation in person living with Duchenne Tool used _____
☐ None

Is the mental health of the individual, caregiver, and family assessed at each visit?

☐ Yes, informally (asking questions during the visit)
☐ Yes, formally Tool Used_____
☐ No

How is ongoing support provided?
☐ Referral to counselors and resources within our institution
☐ Referral to counselors and resources closer to patients/families
☐ None
☐ Other _____

Who in your center discusses neurodevelopmental/psychosocial topics?
☐ Not discussed
☐ Discussed by (choose all that apply)
☐ Neuromuscular clinician
☐ Case manager
☐ Social worker
☐ Neurodevelopmental specialist (neuropsychologist, behavioral pediatrician, etc.)
☐ Nurse, nurse practitioner, nurse coordinator
☐ Other: _____

If neurodevelopmental/psychosocial topics are discussed, which are discussed with parents at least once, and followed up on as appropriate:
☐ Risk for learning disability
☐ Risk for behavioral disorders (ADD/ADHD, autism, OCD)
☐ Need for IEP/504 Plan for cognitive deficits (if appropriate)
☐ Need for IEP/504 Plan for physical disabilities
☐ Need for neurocognitive evaluation (at diagnosis and prior to formal schooling)
☐ Risk for social isolation for child and/or parents
Other _____

Which of the following informal assessments of the person living with Duchenne, are performed at each visit:
☐ Evaluation of emotional adjustment/coping
☐ Speech and language evaluation
☐ Signs of autism spectrum disorders (language delays, repetitive behaviors, deficits in social functioning)
☐ Signs of behavioral disorder (ADD/ADHD, autism, OCD)
☐ Signs of emotional disorder (anxiety, depression)
☐ Signs of social isolation in person living with Duchenne
Other _____

Are social determinants of health for patients and family assessed at each visit ?
☐Yes
☐No
☐Other ____

Age Appropriate Care and Transition

Do patients spend at least a portion of their visit alone with a provider starting in early adolescence (no later than age 14yo)?
☐ Yes
☐ No
☐ Comment
What is the name and title of that provider? _____

All pediatric patients need a transition plan in place by age 14. Please check all elements included in the formal transition plans for your patients
☐ Have a consistent person (care coordinator, nurse or social worker) monitoring & documenting transition plan
☐ Discuss future goals at each visit
☐ Set age appropriate independence goals at every visit
☐ Discuss social and community participation and friendships/relationships at every visit
☐ Begin to assess transition readiness
☐ Discuss and begin developing a plan for transitioning healthcare, education/employment and adult living
☐ Healthcare POA appointed by age 18 years
☐ Advanced care planning, advanced directives, and emergency care plan in place at least by age 18 years

☐ We feel that our program meets the criteria for consideration of certification as defined below:

Duchenne Care Center:

**Certified Duchenne Care Centers must include access to the following providers at each visit: center director, clinic coordination (preferably by a nurse or nurse practitioner), neuromuscular provider, cardiology provider, pulmonary provider, genetic counselor, physical therapist and social worker.

***The following sub-specialty and clinical services must be available as needed for patients/families: gastroenterology/urology, orthopedics, endocrinology, palliative care, surgery, durable medical equipment (venter, distributor), nutrition, orthotics, occupational therapy, psychology/neuropsychology/counseling/developmental pediatrics, speech therapy and wheelchair specialists. All providers must provide care in agreement with the CDC Care Considerations. Coordination of care and appointments, as well as communication to both the primary care provider and family, must be available.

Signature of Center Director	*Date*

Clinical Research Designation

Development of Standards

Significant advancements have been made in the development and implementation of clinical research for potential Duchenne muscular dystrophy therapies. PPMD recognizes centers that demonstrate the standards required to participate in clinical research studies and trials in addition to quality care in an effort to improve transparency so that families and individuals have the necessary information to make informed decisions related to care & research opportunities. Certified Duchenne Care Centers with sufficient clinical trial infrastructure are currently conducting multiple interventional clinical trials and have a strong history of clinical research will be eligible to apply for this additional designation. A center with a Clinical Research Designation will demonstrate excellence in clinical research to the community, highlighting the additional knowledge and expertise within a center, distinct from provisions of clinical care. Designation is granted for a period of five years and will be identified to the Duchenne community through applicable PPMD channels.

Criteria for Centers Applying for Clinical Research Designation

Centers who wish to be considered for Clinical Research Designation must possess the following qualities prior to beginning the certification process:

- Designation as a Certified Duchenne Care Center (CDCC) or actively pursuing CDCC status
- Have an established Duchenne research program at least five years in standing
- Currently conducting at least three interventional clinical trials
- Have established clinical trial infrastructure

Requirements for Research & Services

To be eligible for Clinical Research Designation, a center must be a Certified Duchenne Care Center (CDCC) or actively pursuing certification and this can be requested in parallel. Centers will demonstrate the use of team science and the ability to conduct multidisciplinary research studies for Duchenne muscular dystrophy. Research programs should emphasize knowledge of Good Clinical Practice, DEI (diversity, equity, and inclusion) and contribute to the advancement of the Duchenne community.

Centers must include access to the following providers who should have expertise in Duchenne and Becker muscular dystrophy and collaborate with the clinic/research team.

- Primary Investigator
- Clinical research program lead
- Clinical research coordinators
- Regulatory Coordinator
- Clinical Evaluator trained in Duchenne outcome measures
- Investigational Drug Service
- Cardiologist/ Echotechnician
- Infusion Center

Centers must have access to the following equipment:

- Designated Clinical Evaluator Space (4 Stair Climb, 6MWT,PUL, etc)
- Spirometry
- ECG and Echo
- Centrifuge
- MRI (optional)

Clinical Research Designation Process & Documents

Clinical Research Designation Process

Pathways to obtaining Clinical Research Designation include:

- Current CDCC seeking Clinical Research Designation:
    - Current CDCCs can apply for this designation at the time of their recertification process by completing the additional application. Applications will only be reviewed and granted during the recertification process.

- A new center seeking designation as both a CDCC and Clinical Research Designation:
    - A center seeking both CDCC and Clinical Research Designation will complete the application process for both designations. An in-person site visit is required for all new centers.

For the above pathways, the process to obtain Clinical Research Designation is as follows:

- Inquiries regarding Clinical Research Designation will be made by the requesting center, at which time the CDCC Program Director will engage with the team to assess site qualifications and provide feedback on necessary steps prior to submitting an application.
- Standards for Certification document and application will be sent to the requesting applicant. Once the application is submitted in full, a preliminary assessment will be done by the PPMD care team and/or CDCC Program Director. Supplemental information may be required upon request.
- In addition to submission of the application for Clinical Research Designation, the applicant center must submit:
    - One (1) letter of recommendation from a sponsor describing adherence to good clinical practice, nature of relationship with trial site, and challenges / successes of the site from the sponsor perspective
    - One (1) letter of support from a patient or caregiver describing their experience working with the research team to participate in a clinical trial
- Application will be reviewed by the Certification Committee. If accepted by the committee to move forward, a virtual or in-person site visit will be scheduled (as outlined in following sections)
- The application packet, including site visit summary, letters of support, and post huddle notes will be sent to the certification committee for review and vote.
- If all requirements are met and the certification committee votes for approval, clinical research designation is granted.
- If any outstanding requirements are identified, center leadership is informed and the center will be given 6 months to implement requirements, at which point a virtual check in will be scheduled. If the center is unable to meet requirements within 1 year, they must reapply.
- Once designation is granted, the CDCC Program Director will send necessary documents to the newly certified clinic including notice of award, certification agreement & joint press release
    - Newly designated clinics will agree to announce designation jointly with PPMD
    - Note: This will not be publicly displayed by PPMD until all recertifications for 2025-2026 have taken place and a joint announcement will be made.

Clinical Research Designation Site Visits

Site Visit

At least one member of the Certification Committee and one PPMD care team member will meet virtually or in person with the primary investigator and clinical research lead to review the application. Trial participation

details may be reviewed as part of the virtual pre-site visit (i.e. name of trials, engagement with sponsors, number of participants enrolled). If concerns related to staffing or program structure are identified during the virtual site visit, the in person site visit may be postponed.

Centers Unable to Meet Clinical Research Designation Criteria

Centers who are engaged and committed to excellence in conducting Duchenne clinical trials and research but do not meet full requirements for certification will be given 6 months to implement requirements, at which point a virtual check in will be scheduled. If the center is unable to meet requirements within 1 year, they must reapply.

Should a CDCC with Clinical Research Designation have a change in services available that substantially impacts their qualification for this additional designation, revocation of designation may occur after a review by the certification committee.

Clinical Research Designation Annual Review

Clinical Research Designation will be granted for 5 years, provided that centers:

- Update the Research Services annual document annually and with significant changes to the research program.
- Continue to provide services in alignment with requirements
- Maintain clear and open channels of communication with PPMD and the CDCC Certification Committee

At the end of a 5-year cycle, centers will again be eligible for redesignation.

Clinical Research Designation Renewal

Centers may apply for renewal of Clinical Research Designation every 5 years. To be eligible for renewal, centers must complete the following:

- Complete the online Clinical Research Designation application at time of initial CDCC certification and/or recertification. This includes updating the Research Services Document.
- Need for an in person or virtual recertification site visit will be determined by PPMD staff for CDCC recertification and Clinical Research Designation renewal.
- If an in person site visit is warranted, at least 1 certification committee member and 1 PPMD staff member will shadow in the research center and conduct staff interviews.
- The Certification Committee will review application materials, summary of site visit (if applicable) and vote to recertify.
- Centers must comply with the decisions and recommendations of the Duchenne Care Center Certification Committee, which will be evaluated annually

Clinical Research Designation Application

This will be completed and submitted online via WebGrants

Center information

Name of institution/clinic/center:

Address of institution/clinic/center:

Phone number of clinic/center:

Main Hospital/ institution/ center website:

Director/PI's name (if there is more than 1 director/PI, please list the one most involved):
Name:
Title:
Email:

Address:
Phone:
Fax:

Specialty/subspecialty board certification of the director listed above (choose all that apply):
☐ Pediatrics
☐ Adult, Internal or
☐ Family medicine
☐ Adult neurology
☐ Child neurology
☐ Adult PM&R
☐ Other: _____

Time clinic/center director (named above) has been providing clinical trials for Duchenne muscular dystrophy
☐ 1-5 years
☐ 6-10 years
☐ >11 years

Contact information for families or providers to call for information about clinical trials:
Name:
Title:

Email (internal use only):
Phone Number:

Contact information for families or providers to call with concerns/questions:
Name:
Title:
Email (internal use only):
Phone Number:

How many patients with Duchenne and/or Becker are participating in clinical trials at your center (observational or interventional in the last 5 years)?
☐ Less than 20
☐ 20-50
☐ 50-100
☐ 100-150
☐ 150-200
☐ More than 200

Please list all dystrophinopathy related clinical trials at your site in the past 5 years (industry, grant funded, interventional (device or drug))

Clinical Trial Name	PI Name(s)	# Enrolled	Age criteria	Ambulatory or non-ambulatory	Short description (include years)	Currently enrolling? Yes/no

Please list all dystrophinopathy related observational studies at your site in the past 5 years

Study Name	PI Name(s)	# Enrolled	Age criteria	Ambulatory or non-ambulatory	Short description (include, years,)	Currently enrolling? Yes/no

Have any 483s been issued in the last 5 years?
Yes
No
Other (please explain)

Have any major deviations been reported to the IRB in the last year?
Yes

No

If yes, what was the corrective action plan?

What is the site's largest barrier for participating in research?

What is your diversity, equity and inclusion plan?

Have you been a national PI in the past 5 years?
Yes
No

If yes, what trial?

Do you use a central IRB?

If yes, please list IRBs that can be used

On average, how long does study start up take?

On average, how long does it take to obtain IRB approval?

On average, how long does it take to receive a finalized contract?

Is your center able to undergo IRB review and contracting simultaneously?

Are you or any of your team members consulting for any companies currently or in the past 3 years?
Yes
No

If yes, please specify which team members, the consulting company, and their role

Please list any publications, posters or presentations related to research completed at your site in the last 3 years. Only include if the team member is the primary or secondary author. You may drop your CV, biosketch or other supporting documents.

Title	Date	Category	Short Description

☐ We feel that our program meets the criteria for consideration of certification as defined below:

Clinical Research Designation:

- Certified Duchenne Care Centers with a Clinical Research Designation must have sufficient clinical trial infrastructure, are currently conducting at least 3 interventional clinical trials and have a strong history of duchenne clinical research at least five years in standing. These centers demonstrate the use of team science and the ability to conduct multidisciplinary research studies for Duchenne muscular dystrophy. Research programs should emphasize knowledge of Good Clinical Practice, DEI (diversity, equity, and inclusion) and contribute to the advancement of the Duchenne community. Centers with a Clinical Research Designation must include access to a Primary

Investigator, Clinical research program lead, Clinical research coordinators, Regulatory Coordinator, Clinical Evaluator trained in Duchenne outcome measures, Investigational Drug Service, Cardiologist/ Echotechnician, and Infusion Center all of whom have expertise in Duchenne and Becker muscular dystrophy and collaborate with the clinic/research team.

Signature of Center Director	*Date*

CERTIFICATION MAINTENANCE

In order to maintain certification, Certified Duchenne Care Centers will agree:

- To the public sharing of certification status.
- That only pertinent clinic and contact information collected from Certified Duchenne Care Centers will be shared publicly on the PPMD website
- That comments and suggestions made by the Certification Committee will not be made public, and will be shared only with the applicant(s) and followed up annually.
- That evaluations and comments made by patients/parents on the Patient Experiences surveys will not be made public, and will be shared only with the Certification Committee and individual centers.
- That information regarding care, services, clinical research and staffing will be provided by the centers and will be kept current (i.e., updated at least annually through the Annual Report process)
- Update PPMD within eight weeks about any changes in core services or providers at the center
- Annual reporting of key data elements as identified in annual report process including but not limited to number of patients served, patient demographics, trial participation and/or referral process
- To have a mechanism in place to gather patient experiences in the clinical or research program through the patient experience survey provided by PPMD
- To commit to data sharing to enhance knowledge and care for patients with dystrophinopathy
- That at least one clinic member will attend PPMD's Duchenne Healthcare Professionals Summit and/or Advocacy Conference and/or Annual Conference annually
- Consider participation in specialty workgroup, projects, and/or initiatives as they arise
- To maintain clear and open channels of communication with PPMD and the Certification Committee

Revocation of Certification

If the Certification Committee determines that the requirements to maintain certification are not being met, the Committee may revoke certification. Examples of reasons a certification may be revoked include, but are not limited to the following situations:

1. Care provided is no longer aligned with the CDCC Program Standards
2. Required annual information has not been provided
3. Turnover in staff has changed the quality or standard of care being provided at the center
4. Team members have not attended annual meetings, including the Healthcare Professionals Summit, Advocacy or Annual Conference
5. Research program no longer actively conducting clinical trials due to staff turnover, institutional support, or other reasons
6. Research program has multiple protocol deviations or safety events calling into question consistency and competency in ability to conduct clinical trials

Centers will receive recommendations and guidance from the Certification committee regarding required information needed or changes in the provision of care, and be given a period of 6-12 months to meet the requirements. If the requirements are met to the satisfaction of the Certification Committee within 6-12 months, the center will maintain certification. If requirements are not met in 6-12 months, certification will be revoked and the center will be required to reinitiate the certification process.

List of Certified Duchenne Care Centers

A list of Certified Duchenne Care Centers may be found on the PPMD website with indication of Centers with Clinical Research Designation.

Glossary

Certification: demonstrates commitment to excellence in providing Duchenne-specific care and management in a comprehensive, standardized manner

Certified Duchenne Care Center Program (CDCC Program): a program of Parent Project Muscular Dystrophy to identify centers capable of providing comprehensive care and clinical services to people living with Duchenne and to communicate that knowledge to the Duchenne community, including patients, families, caregivers, national organizations, private and publicly-held companies, government institutions, industry and other key stakeholders.

Certified Duchenne Care Center (CDCC) - A Certified Duchenne Care Center demonstrates commitment to excellence in providing Duchenne-specific care and management in a comprehensive, standardized manner. Certification of Duchenne Care Centers will improve the quality of patient care by standardizing care and, therefore, reducing discrepancies in care across the United States.

Clinical Research Designation - A Certified Duchenne Care Center that demonstrates a commitment to excellence in the provision of dystrophinopathy clinical trials and research. Centers with a Clinical Research Designation have sufficient clinical trial infrastructure, are currently conducting multiple interventional clinical trials and have a strong history of clinical research.A Clinical Research Designation will improve transparency and access to research and potential treatments and therefore reduce discrepancies in care and trial participation across the United States.

CDCC Program Certification Committee: this committee will conduct the process of certification. Responsibilities of the CDCC Program Certification Committee include review of applications submitted by centers applying for certification, participation in virtual meetings with the applying centers to discuss the application and certification process, site visits of applicant centers (at least 1 members of the Certification Committee will attend each site visit) including interviews of faculty and staff and patient records review

DMD Care Considerations Working Group (DMDWG): an effort of the Centers for Disease Control and Prevention (CDC), which published the CDC Care Considerations, standards of Duchenne care which were updated and published in 2018). Members of this Core Group included experts in sub-specialty Duchenne care from across the United States.

Duchenne Registry: a PPMD maintained registry, mobile application, and website, whose purpose is to collect patient reported outcomes, to connect people living with Duchenne, with actively recruiting clinical trials and research studies and to educate people and families about Duchenne research

Parent Project Muscular Dystrophy (PPMD): a national organization, whose mission is research, advocacy, care and education for patients and families living with Duchenne muscular dystrophy.

Standards: requirements of certification as a Duchenne Care Center, which include providing care that is aligned with the standards of care described by the DMD Care Considerations Working Group (DMDWG). The DMD Care Considerations were published the CDC Care Considerations and were updated and published in 2018

Transforming Duchenne Care Initiative (TDCI): an effort by PPMD to minimize differences in the care and management of people living with Duchenne in the United States.