MEASURE #1: Patients with DMD Prescribed Appropriate Disease Modifying Pharmaceutical Therapy

MUSCULAR DYSTROPHY

Measure Description
All patients diagnosed with Duchenne muscular dystrophy (DMD) prescribed appropriate DMD disease modifying pharmaceutical therapy*.

Measure Components

<table>
<thead>
<tr>
<th>Numerator Statement</th>
<th>Patients prescribed appropriate DMD disease modifying pharmaceutical therapy*.</th>
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*Current appropriate disease modifying pharmaceutical therapy for DMD: Corticosteroids

<table>
<thead>
<tr>
<th>Denominator Statement</th>
<th>All patients diagnosed with Duchenne muscular dystrophy (DMD).</th>
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</thead>
</table>

Exceptions:
- Medication exception for not prescribing disease modifying pharmaceutical therapy (i.e., medical contraindication; patient already on corticosteroid; may not be medically appropriate depending upon functional capability, age, and existing risk factors)
- Patient exception for not prescribing disease modifying pharmaceutical therapy (i.e., patient or family caregiver declines)
- System exception for not prescribing disease modifying pharmaceutical therapy (i.e., patient has no insurance to cover prescription and cannot afford it)

Supporting Guideline & Other References
- Treatment with corticosteroids to prevent the development or progression of scoliosis in DMD patients may be considered, even if the patient is wheelchair-bound.¹
- It is recommended to follow the Dutch guidelines for the usage of corticosteroids in DMD patients.¹
- Benefits and side effects of corticosteroid therapy need to be monitored. Timed function tests, pulmonary function tests, and age at loss of independent ambulation are useful to assess benefits. An offer of treatment with corticosteroids should include a balanced discussion of potential risks. Potential side effects of corticosteroid therapy include weight gain, cushingoid appearance, cataracts, short stature (i.e., a decrease in linear growth), acne, excessive hair growth, gastrointestinal symptoms, and behavioral changes also need to be assessed. If excessive weight gain occurs (20% over estimated normal weight for height over a 12-month period), based on available data, it is recommended that the dosage of corticosteroids be decreased (to 0.5 mg/kg/day with a further decrease after 3 to 4 months to 0.3 mg/kg/day if excessive weight gain continues). (Level A)²
- Deflazacort (0.9 mg/kg/day) can also be used for the treatment of DMD in countries in which it is available. (Level A)²
- Prednisone has been demonstrated to have a beneficial effect on muscle strength and function in boys with DMD and should be offered (at a dose of 0.75 mg/kg/day) as treatment. (Level A)²
- On the basis of this convincing literature and practice parameter guidelines, the panel strongly urges consideration of glucocorticosteroid therapy in all
patients who have DMD. (Formal Consensus Statement)\(^3\) The goal of the use of glucocorticoids in the ambulatory child is the preservation of ambulation and the minimization of later respiratory, cardiac, and orthopedic complications, taking into account the well-described risks associated with chronic glucocorticoid administration. If such issues are pre-existing, the risk of side-effects might be increased. Particular care needs to be taken with such patients in deciding which glucocorticoid to choose, when to initiate treatment, and how best to monitor the child for any problems. A high index of suspicion for steroid-related side-effects needs to be maintained at all times. Prevention and management of side-effects needs to be proactive. Families should be provided with a steroid card or similar notification that the child is on steroids, listing emergency-care considerations in the setting of acute medical presentation, fracture, serious infection, need for surgery, or general anesthesia, to alert any medical professional with whom the child might come into contact.\(^3\)

- Open discussion across the multidisciplinary team regarding the type and duration of specific interventions encourages transparency and shared decision-making.\(^4\)

- Current recommendations indicate that the timing of initiation of glucocorticoid therapy must be individualized, considering the functional capabilities, age, and preexisting risk factors for adverse effects in each child. Initiation of glucocorticoids is not recommended for a child who is still gaining motor skills, which usually plateaus around the ages of 4-to-8 years. The child who takes longer to perform motor tasks in timed testing, loses a skill (such as climbing stairs), shows less endurance, or has more falls should be considered for starting glucocorticoid therapy.\(^5\)

- Some variability about the dosing of glucocorticoids exists. The majority of available evidence indicates that glucocorticoids should be given in a single daily dose, commonly oral prednisone at a starting dose of 0.75 mg/kg/d. A recent study comparing daily prednisone (0.75mg/kg/d) versus high-dose (10 mg/kg/d) weekend prednisone demonstrated equal benefits and overall good tolerability of both dosing regimens. Another study compared daily doses of prednisone to alternate-day dosing; the results were that those boys assigned to the alternate-day therapy had significant loss of strength by 3 months compared to those on the daily dose regimen. Boys on the daily dose regimen did not lose strength for the duration of the study. Furthermore, no major differences in adverse effects were seen between the two groups.\(^10\) If adverse effects such as weight gain require a decrease in dose, then a gradual tapering to dosages as low as 0.3 mg/kg/d may still be beneficial.\(^5\)


Rationale for the Measure

Gap in Care
DMD is a recessive X-linked genetic disorder characterized by progressive muscle weakness and reduced muscle tone. Affecting only boys, it limits life expectancy to approximately 20 years. Care for patients with DMD is poorly standardized. This leads to inequality in access to treatment.¹

Although there is no cure, a Cochrane Review and AAN practice parameter concluded that prednisone may provide short term effective treatment that prolongs the ability to walk, reduces the complications such as scoliosis, respiratory insufficiency and cardiac impairment. Despite the well documented beneficial effects of corticosteroids in DMD, a population based study of corticosteroid use between 1991 and 2005 reported that only 50.9% of individuals had ever been on corticosteroids. The annual mean percent corticosteroid use varied widely from 8.4% to 80.2% across clinics.² Another survey showed that nearly 10% of neuromuscular disease clinics do not offer such therapy.³

Glucocorticoids are currently the only medication available that slows the decline in muscle strength and function in DMD, which in turn reduces the risk of scoliosis and stabilizes pulmonary function.⁴ Approximately 16% of Muscular Dystrophy Association clinic directors report not using corticosteroids.³

Opportunity for Improvement
The goal of the use of glucocorticoids in the ambulatory child is the preservation of ambulation and the minimization of later respiratory, cardiac, and orthopedic complications.³ Studies have shown that providing corticosteroid treatment early, such as in 2-to-4 year old DMD patients, can prolong the ability to walk, slow down respiratory decline, and preserve left ventricular ejection fraction.⁵ ⁶ There is also data to support the longer term (>3 years) use of corticosteroids to prolong ambulation, reduce the need for spinal stabilization surgery, improve cardiopulmonary function, delay the need for non-invasive ventilation, and improve quality of life and survival in patients with DMD.⁷

This quality measure has the opportunity to reduce the risk of scoliosis, stabilize pulmonary function, and potentially delay decline in respiratory and cardiac function.

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<thead>
<tr>
<th>Type of measure</th>
<th>Process</th>
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<tbody>
<tr>
<td>Level of Measurement</td>
<td>Individual practitioner</td>
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<tr>
<td>Care setting</td>
<td>Outpatient visits</td>
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<td>Nursing homes</td>
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<tr>
<td>Data source</td>
<td>Electronic health record (EHR) data</td>
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<td>Data registry</td>
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**Technical Specifications: Electronic Health Record/Registry (Under Development)**

Administrative claims data collection requires users to identify the eligible population (denominator) and numerator using codes recorded on claims or billing forms (electronic or paper). Unfortunately, DMD is not identifiable by an ICD-9 or ICD-10 code; rather it is grouped together with several other muscular dystrophy codes under one code (ICD-9: 359.1; ICD-10: G71.0 Muscular dystrophy). Therefore, the work group felt that this measure should be focused only on electronic health records and registries where the specific type of muscular dystrophy, DMD, can be easily identified. There is a SNOMED-CT code for Duchenne muscular dystrophy (disorder) Concept ID: 76670001 but this coding system is not commonly used for claims in the United States currently.

**Coding**

EHR or Registry diagnosis code of Duchenne Muscular Dystrophy. Full code value sets, logic and eMeasure HL7 format under development.

**Denominator**

SNOMED-CT Code for Duchenne muscular dystrophy (disorder) Concept ID: 76670001