

Supplemental Information

GLUCOCORTICOID MANAGEMENT

The management of DMD varies by clinical course. In young children, especially those who present by the age of 1 year, clinicians can consider initiating glucocorticoids in congenital and infantile forms of DMD. Connolly et al²¹ have shown that the Bayley Scales of Infant Development, Third Edition is a useful tool for assessing functional measures in infants. Live-attenuated vaccines can be administered to infants on glucocorticoids, although the risk of immunosuppression with the measles-mumps-rubella and varicella vaccines should be taken into account in infants on chronic glucocorticoids. The Centers for Disease Control and Prevention guidelines allow for glucocorticoid doses of <2 mg/kg in young patients. Therefore, the administration of glucocorticoids should not prevent infants and children from receiving live vaccines, although close monitoring should occur during this period of time.

In children with classic presentations between the ages of 3 and 5 years, the general approach has been to serially monitor the children

for signs of muscle weakness and start glucocorticoids at that time, which usually occurs around the time immunizations have been completed. Identifying early signs of proximal muscle weakness and initiating glucocorticoids early can further prolong ambulation. Early treatment can minimize skeletal fibrosis.^{8,92} However, recently, Kim et al⁹³ reported an associated risk of cardiomyopathy, bone fracture, and decreased pulmonary function with the initiation of glucocorticoids in early childhood.

Glucocorticoids are typically continued into adolescence. There is emerging evidence with which researchers support continuing glucocorticoids into adulthood with benefits for muscles used in respiration, upper extremities, and a mild cardioprotective effect in patients aged ≥ 20 years.^{94,95} Their long-term use into adulthood varies widely, primarily because of each individual's tolerance of the steroids. Often, behavior and weight gain are the most common reasons leading to dose adjustments to limit undesired side effects or to discontinuation. Equally important are the risks of osteoporosis, bone fracture, and

adrenal insufficiency (for additional details, see the specialty article on osteoporosis management⁹⁶ that is part of this supplement and the 2018 Duchenne Muscular Dystrophy Care Considerations Parts 1 and 2, which include guidelines for managing endocrine⁹⁷ and osteoporosis¹⁷ issues).

In nonambulatory teenagers and young men, glucocorticoids may have benefits for cardiopulmonary function.^{13,23,24} At this stage, the care strategy is to optimize upper-extremity function, pulmonary care, and the management of cardiomyopathy and/or fibrosis¹⁷ (for additional details, see the specialty articles on respiratory⁹⁸ and cardiology care¹⁶ that are part of this supplement). Furthermore, a consideration of pain management is included in the 2018 Care Considerations as part of a comprehensive care approach along with palliative and psychosocial support⁹⁹ (for additional details, see the specialty article on psychosocial management that is part of this supplement¹⁰⁰).

SUPPLEMENTAL REFERENCES

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