Recommendation for the Addition of Duchenne Muscular Dystrophy (DMD) to the Iowa Newborn Screening Panel

Iowa Council for Health and Human Services Ad Hoc Committee for Management of the Newborn Screening Panel

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Duchenne Muscular Dystrophy (DMD)

DMD affects about 2 per 10,000 newborn males (for Iowa, this would be about 5-7 live-born males a year), leading to progressive weakness and premature death, typically from respiratory or cardiac complications. Abnormal gait and falls are usually the first symptoms, occurring in the preschool years. The average age of diagnosis in the United States (US) over the past four decades has been 4.5 to 5 years.

The availability of targeted therapies and the long diagnostic odyssey have led to advocacy for newborn screening (NBS). The Secretary of the US DHHS added DMD to the Recommended Uniform Screening Panel (RUSP) in December of 2025.

DMD newborn screening is based on measuring creatine kinase (CK-MM), which is elevated due to muscle damage. Infants with borderline CK-MM levels (often attributed to birth trauma) can be retested after at least one week to determine if elevations are birth-related.

Kemper AR, Lam WKK, Ojodu J, et al. Evidence Regarding Duchenne Muscular Dystrophy Newborn Screening. *Pediatrics.* 2025; doi: 10.1542/peds.2025-073192

DMD Treatments

Non-pharmacologic approaches, including genetic counseling, physical therapy, neurocognitive therapy, optimization of nutrition, respiratory support, and orthopedic appliances, are important in the care of patients with DMD, and are appropriate at an early age.

Historically, steroids, such as prednisone, have been the main pharmacologic standard of care. They reduce DMD-related muscle damage, stabilize cell membranes, and promote muscle fiber growth. A 2016 systematic review supported the benefit of glucocorticoids in improving muscle strength and other functional outcomes but was unable to evaluate the impact of presymptomatic or early symptomatic intervention. Long-term use of prednisone at standard doses is associated with significant adverse effects (e.g., decreased linear growth, osteoporosis, fractures, weight gain, cataracts, behavior change). Although treatment with glucocorticoids or related medications can improve outcomes for DMD despite side effects, the optimal age of initiation is unclear – Dr. Katherine Mathews, physician and professor of pediatrics in neurology at the University of Iowa Stead Family Department of Pediatrics, states she starts steroids and other therapies at around 3-4 years of age.

Efficacy of Food and Drug Administration-approved gene therapy has not been established.

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Ad Hoc Committee Review

The Ad Hoc Committee met in October 2026 to conduct an evidence-based review of DMD for consideration of adding it to Iowa's newborn screening panel, using an established review framework.

Committee members examined peer-reviewed literature and information from other state newborn screening programs screening for DMD and heard a presentation from a pediatric neuromuscular physician experienced in providing care for children with neuromuscular disorders, such as DMD. Iowa Newborn Screening Program (INSP) staff spoke to the capacity of the INSP to screen for MLD, and a parent of two sons with DMD testified as to their lived experience.

Parallel to this review, Kimberly Noble Piper met with Iowa Medicaid partners to update them on the current newborn screening panel and the potential addition of DMD, including treatment options and the possible cost of treatments.

Benefits and Harms

- The benefits to newborn screening for DMD are that it permits early identification of the condition so parents may avoid the diagnostic odyssey (often children aren't diagnosed until 4-5 years of age), and newborns, families and providers benefit from early intervention services. The provider may monitor the newborn and start treatments as early as 3 years of age. Knowing a child's DMD diagnosis early can inform future pregnancies (by the time the older child is diagnosed, the parents may have already had other children).

- If a baby is not screened for DMD, diagnosis of the condition is delayed until the child demonstrates symptoms, which may take years to diagnose. This delays treatments and therapies that may mitigate the effects of the condition, and parents may have had another child before the older child is identified with DMD.

The Committee recognizes that while clinical treatments such as steroids are not indicated for newborns, the benefit of early interventions such as therapy and genetic counseling are beneficial if accessed as a newborn or infant.

Ad Hoc Committee Recommendation

After discussion and deliberation, the Ad Hoc Committee reached consensus on a recommendation to add DMD to Iowa's newborn screening panel.

The Committee puts forth a Level B2 Recommendation:

"Newborn screening for Duchenne muscular dystrophy has a moderate certainty of clinical net benefits and a high certainty of the net benefit of early intervention therapies for newborns, families and providers; and DMD newborn screening has high feasibility. INSP has developmental readiness to screen within 18 months."