Congress of the United States

Washington, DC 20515

November 25, 2025

The Honorable Robert F. Kennedy, Jr.
Secretary
U.S. Department of Health and Human Services
200 Independence Avenue SW
Washington, D.C. 20201

Dear Secretary Kennedy:

We write to express our strong support for adding Duchenne Muscular Dystrophy (Duchenne) to the Recommended Uniform Screening Panel (RUSP).

Duchenne is a fatal genetic disorder that affects approximately one in every 5,000 male births. This devastating, progressive disease begins damaging boys' muscles by the time they are born, as evidenced by elevated creatine-kinase (CK)-MM in their blood. Unfortunately, most families are unaware of their child's condition until after the age of four, following years of irreversible muscle damage and loss.

Currently, a diagnosis of Duchenne following a lengthy diagnostic journey results in missed opportunities for care, for accessing clinical trials, and for initiating treatment. Newborn screening ensures that diagnosis is not dependent on where a child is born, on its family resources, or its provider's awareness—giving families time to plan and implement care strategies. Newborn screening for Duchenne also allows these boys to seek interventions during an optimal window before muscle loss occurs. The earlier the intervention, the more effective it can be.

Duchenne is detectable and clinically present at birth. Families deserve to know their son's diagnosis early and definitively so that they can slow disease progression and improve quality of life by accessing meaningful clinical interventions and U.S. Food and Drug Administration (FDA)-approved therapies. Coordinated multidisciplinary care, including physical therapy, occupational therapy, and guidelines for physical exercise, can be specifically tailored to support children with Duchenne. Comprehensive standards of care are well established and can be initiated promptly following diagnosis.

FDA-approved therapies are available, and earlier access improves outcomes. Currently, corticosteroids and exon-skipping therapies are available to children younger than the mean age of diagnosis. Other treatment modalities, such as gene therapies, are soon likely to be available at younger ages. Science is increasingly showing us that the key to extended life spans and

improved quality of life for boys with Duchenne hinges on interventions at the earliest age possible.

Early identification will positively change the lives of affected children and families, while reducing the long-term health and economic burden on states and the health care system. In addition, newborn screening has been shown to accelerate therapeutic development, potentially leading to safer and even more effective therapies arriving at a more rapid pace.

Early diagnosis is transformational and simple to achieve. Duchenne can be reliably detected through established and validated newborn screening methods. CK-MM blood testing, which is FDA-approved, is the standard approach for screening for the muscle damage caused by Duchenne. Multiple pilot studies tested CK-MM over several decades, and it is used in Ohio and Minnesota's newborn screening programs. Furthermore, utilizing CK-MM as the newborn screen, with genetic testing as the confirmatory test, should minimize false positives and false negatives while successfully diagnosing infants with Duchenne. Over 40 state newborn programs already have access to the equipment necessary to screen for Duchenne.

Adding Duchenne to the RUSP reflects an achievable commitment to early, timely diagnosis. It is the next critical step in ensuring that every child born with Duchenne has the best chance at a longer, healthier life. We urge the Department of Health and Human Services and the Health Resources and Services Administration (HRSA) to recommend and approve the addition of Duchenne muscular dystrophy to the RUSP.

Sincerely,

Roger P. Wicker
United States Senator

Amy Klobuchar
United States Senator

Troy Balderson
Member of Congress

David P. Joyce
Member of Congress

Michael Guest
Member of Congress

Frank J. Mrvan
Member of Congress

Robert B. Aderholt
Member of Congress

Pete Stauber
Member of Congress

Christopher A. Coons
United States Senator

Susan M. Collins
United States Senator

Cc:
Thomas Engels, Administrator, Health Resources and Services Administration
Laura Kavanagh, MPP, Acting Associate Administrator, Maternal and Child Health Bureau, Health Resources and Services Administration
CDR Leticia Manning, MPH, Newborn Screening Team Lead, Division of Services for Children with Special Health Needs, Maternal and Child Health Bureau, Health Resources and Services Administration