

**ORANGE COUNTY BOARD OF HEALTH
AGENDA ITEM SUMMARY**

Meeting Date: Wednesday, January 21, 2026

Agenda Item Subject: Youth for Rare Diseases and Accelerating Newborn Screening for Metachromatic Leukodystrophy (MLD) and Duchenne Muscular Dystrophy (DMD) in North Carolina

Attachment(s): Youth for Rare Diseases and Accelerating Newborn Screening for Metachromatic Leukodystrophy (MLD) and Duchenne Muscular Dystrophy (DMD) in North Carolina.PDF

Staff or Board Member Reporting: Vrinda, Youth For Rare Diseases Nonprofit Founder

Purpose: Action
 Information only
 Information with possible action

Summary Information:

Vrinda is the founder of a youth-led nonprofit called Youth For Rare Diseases which aims to advocate for families affected by rare diseases which affect 1 in every 10 people. Youth For Rare Diseases is specifically working to raise awareness for an ultra-rare and fatal disorder known as Metachromatic Leukodystrophy (MLD). In November 2025, fifteen local youth volunteers completed a public health initiative to raise awareness for MLD, gaining about 500 handwritten signatures from North Carolina residents supporting the addition of MLD to our state's newborn screening panel.

Though MLD and a similar rare disease, Duchenne Muscular Dystrophy (DMD), were added to the Federal Recommended Universal Screening Panel (RUSP) in December of 2026, the addition of MLD and DMD to the North Carolina newborn screening panel is still awaiting State approval. State regulations respond to local priorities, and county leadership decisions can affect state timelines. Community effort and support will help emphasize the importance and urgency of the cause to NC legislators.

Health Equity Impact:

Ensuring all newborns in North Carolina have the same screening panel, including Metachromatic Leukodystrophy (MLD) and Duchenne Muscular Dystrophy (DMD), will improve health outcomes for those affected by these rare and serious disorders. By raising awareness of and potentially advocating for the inclusion of MLD and DMD on the NC Newborn Screening Panel, early intervention can be improved and help reduce the complications and severity of symptoms for individuals born with these conditions.

Recommended Action: Approve
 Approve & forward to Board of Commissioners for action
 Approve & forward to _____
 Accept as information
 Revise & schedule for future action
 Other (detail): Accept as information and consider taking action to support the cause in the future (e.g., through writing a letter of support or resolution).

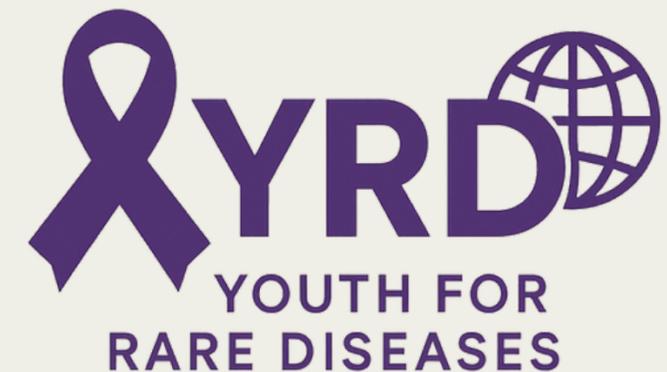
Accelerating Newborn Screening for MLD and DMD in North Carolina

RESOLUTION REQUEST FROM ORANGE COUNTY

Youth For Rare Diseases Team,
Presenter/President- Vrinda,
Vice Presidents- Melody, Nikhila, Kiran, Srinidhi, Madison



For some diseases, time
lost is function lost.



WHAT IS METACHROMATIC LEUKODYSTROPHY

- Rare, inherited neurometabolic disorder
- Children look healthy at birth but symptoms typically begin after brain damage has already occurred
- FDA-approved gene therapy exists and is most effective before symptoms start

WHAT IS DUCHENNE MUSCULAR DYSTROPHY

- Rare, inherited progressive muscle disorder
- Children appear healthy at birth but diagnosis often occurs after muscle damage has already begun (at age 4-5 typically)
- Eight FDA-approved therapies exist and many with better outcomes when started before symptoms occur

Clinical Diagnosis comes too late.



SCIENCE IS READY

- FDA-approved therapies exist for both MLD and DMD
- Early treatment significantly improves outcomes
- Screening assays are validated

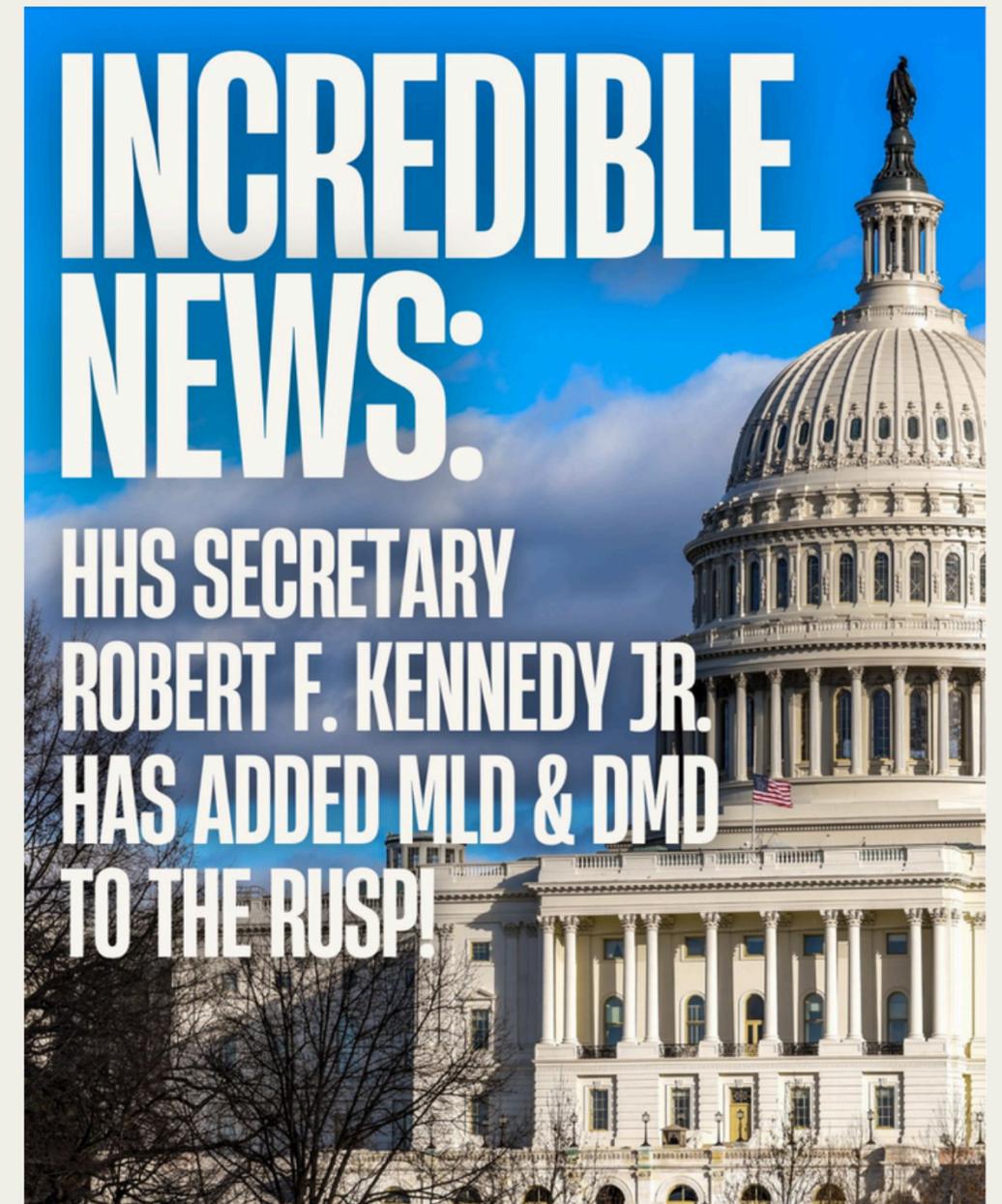
FEDERAL RECOMMENDATION

On December 16 2025, MLD and DMD were officially added to the Federal Recommended Universal Screening Panel also known as RUSP.

North Carolina now has 3 years to implement screening for MLD and DMD following RUSP recommendation.

Delays can still happen despite the 3 year implementation goal.

Sources: ParentProject MD



NATIONAL MOMENTUM

DMD Pilots: New York, Massachusetts, North Carolina

MLD: New York, Washington State

THE PAST DELAY: POMPE

Added to recommended universal screening panel (RUSP): 2015

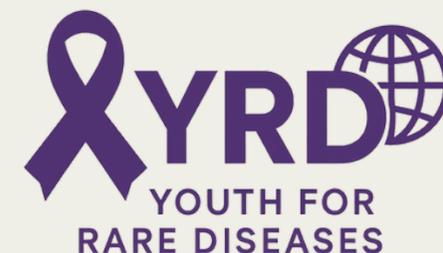
FDA Approved enzyme (myozyme) replacement therapy already existed

It was developed by researchers at Duke University

North Carolina began Pompe screening: March 2023

8-year delay

Sources: Duke Health, HRSA, NCDHHS



NORTH CAROLINA IS ALREADY BUILT FOR THIS

Early Check Pilot: 16,000 NC babies screened for DMD already.

Clinical experts: Home to UNC Chapel Hill and Duke University

Community Push: NC implemented Pompe and MPS I after collective effort.

WHY LOCAL ACTION MATTERS

State systems respond to local priorities

County leadership decision affect state timelines

Community effort will emphasize the importance and urgency of this cause

Local Support shapes state's speed which is exactly what's needed

Specifically,

Help Secure Early funding and Enforce the 18 month timeline

WHY WE ARE ASKING FOR YOUR SUPPORT

Your resolution of support would be the first step towards getting leaders across the state to push for getting MLD and DMD screening done faster so that no newborns are missed and delays are prevented.

Thank you

If you have any questions, please feel free to ask!

Vrinda

Links to Sources-

<https://my.clevelandclinic.org/health/diseases/6067-metachromatic-leukodystrophy>

<https://www.health.state.mn.us/news/pressrel/2025/newborn050925.html>

<https://www.health.state.mn.us/news/pressrel/2025/newborn050925.html>

<https://www.parentprojectmd.org/care/approved-therapies-for-duchenne-muscular-dystrophy/>

<https://www.health.state.mn.us/news/pressrel/2025/newborn050925.html> <https://www.parentprojectmd.org/care/approved-therapies-for-duchenne-muscular-dystrophy/>

<https://medschool.duke.edu/stories/duke-continues-take-extraordinary-measures-treat-pompe-disease>

[https://www.health.ny.gov/press/releases/2025/2025-11-](https://www.health.ny.gov/press/releases/2025/2025-11-12_newborn_screening_for_mld.htm#:~:text=You%20are%20Here:%20Home%20Page,newborn%20screening%20for%20this%20condition.)

[12_newborn_screening_for_mld.htm#:~:text=You%20are%20Here:%20Home%20Page,newborn%20screening%20for%20this%20condition.](https://www.health.ny.gov/press/releases/2025/2025-11-12_newborn_screening_for_mld.htm#:~:text=You%20are%20Here:%20Home%20Page,newborn%20screening%20for%20this%20condition.)

https://sboh.wa.gov/sites/default/files/2025-11/Tab11b-NBSPetition-MLD-SBOHPresentation-Nov2025_0.pdf