WASHINGTON STATE BOARD OF HEALTH

Date: November 19, 2025

To: Washington State Board of Health Members

From: Kelly Oshiro, Vice Chair

Subject: Petition for Rulemaking – Chapter 246-650 WAC, Newborn Screening, Request to Add Metachromatic Leukodystrophy (MLD)

Background and Summary:

The Administrative Procedures Act (RCW 34.05.330) allows any person to petition a state agency to request the adoption, amendment, or repeal of any rule. Upon receipt of a petition, the agency has sixty days to either (1) deny the petition in writing, stating the reasons and, as appropriate, offer other means for addressing the concerns raised by the petitioner, or (2) accept the petition and initiate rulemaking.

On October 6, 2025, the Washington State Board of Health (Board) received a petition to amend chapter 246-650 WAC to add Metachromatic leukodystrophy (MLD) to the newborn screening panel. Under RCW 70.83.050, the Board is responsible for establishing rules for the Newborn Screening program within the Department of Health (Department). The Board's rules are set forth in chapter 246-650 WAC and include the list of conditions that all newborns must be screened for.

The petition notes that MLD screening is feasible on existing multiplexed equipment, highly reliable, and has shown no false positives or negatives in pilot studies of over 500,000 newborns. In addition, it states several states, including New York, Illinois, Minnesota, and Pennsylvania, are implementing or planning population-wide MLD screening. The petition also emphasizes that early identification is critical to ensure timely treatment, as therapy is most effective before symptoms appear.

According to published literature, MLD is a rare inherited lysosomal storage disorder caused by mutations in the arylsulfatase A (ARSA) gene. Mutations in this gene lead to reduced enzyme activity and accumulation of sulfatides in the brain and nervous system.[1,2] The condition affects approximately one in 100,000 newborns and is progressive and fatal without treatment.

Based on available published research, gene therapy (arsa-cel) has shown improved outcomes when administered early, particularly for infants with late-infantile and early juvenile forms of MLD.[2] Because most affected infants have no family history, newborn screening offers the best opportunity for early detection and intervention. MLD is currently under review by the Health Resources and Services Administration (HRSA) for possible inclusion on the federal Recommended Uniform Screening Panel (RUSP).[3] Previously, it was being evaluated by HRSA's Advisory Committee on Heritable Disorders in Newborns and Children (ACHDNC), which was terminated by the U.S. Department of Health and Human Services (HHS). The American Academy of Pediatrics published an evidence-based review of MLD in August 2025, and HRSA opened a public comment period, which closed on September 15, 2025. To date, no updates have been issued on the outcome of the HRSA review.

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The Board relies on a multi-disciplinary technical advisory committee (TAC) to evaluate candidate conditions using established criteria and guiding principles. Before convening a TAC, the Board must first determine whether sufficient scientific evidence exists to apply its inclusion criteria. The Board recently clarified in its process document that if a condition is included on the RUSP, this evidence requirement may be considered met, allowing a TAC review to proceed without a full Board briefing to decide whether to convene a TAC.

I have invited Molly Dinardo, Board staff, and Megan McCrillis, Department staff, to provide an overview of the Board's process for adding a condition to the panel, the petition request, and a summary of MLD and the federal review.

Recommended Board Actions:

The Board may wish to consider and amend, if necessary, one of the following motions:

The Board accepts the petition for rulemaking, submitted under RCW 34.05.330, to amend chapter 246-650 WAC to add Metachromatic leukodystrophy (MLD) as a condition for newborn screening. The Board directs staff to notify the petitioner of this decision and file a CR-101 to initiate rulemaking under the Board's authority in RCW 70.83.050.

OR

The Board declines the petition for rulemaking to add Metachromatic leukodystrophy (MLD) as a condition for newborn screening in chapter 246-650 WAC. The Board directs staff to work with the Department of Health to convene a technical advisory committee to evaluate MLD using the Board's established process and criteria for evaluating conditions for the Washington State newborn screening panel, and to provide their recommendation to the Board.

OR

The Board declines the petition for rulemaking to add Metachromatic leukodystrophy (MLD) as a condition for newborn screening in chapter 246-650 WAC. The Board directs staff to work with the Department of Health to monitor the outcome of the Health Resources Services Administration Recommended Uniform Screening Panel review of MLD and report back to the Board so the Board can then consider whether to establish a technical advisory committee to evaluate MLD for possible inclusion in the Washington State newborn screening panel.

Staff

Molly Dinardo, Policy Advisor

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[1] U.S. National Institutes of Health, Genetic and Rare Diseases Information Center. Metachromatic leukodystrophy. September 2025. Accessed October 29, 2025. https://rarediseases.info.nih.gov/diseases/3230/metachromatic-leukodystrophy
[2] Wendy K.K. Lam, Margie A. Ream, Scott D. Grosse, Jelili Ojodu, Elizabeth Jones, Hadley Stevens Smith, Anne Marie Comeau, Susan Tanksley, Alex Kemper; Evidence Regarding Metachromatic Leukodystrophy Newborn Screening. Pediatrics 2025; 10.1542/peds.2025-073188; https://doi.org/10.1542/peds.2025-073188
[3] U.S. Department of Health and Human Services, Health Resources and Services Administration. Notice with request for comment: Consideration of adding metachromatic leukodystrophy to the Recommended Uniform Screening Panel (90 FR 39196). August 14, 2025. Accessed October 29, 2025. https://www.federalregister.gov/documents/2025/08/14/2025-15432/notice-with-request-for-comment-consideration-of-adding-metachromatic-leukodystrophy-to-the