



## **DEPARTMENT OF HEALTH AND HUMAN SERVICES**

### **Health Resources and Services Administration**

#### **Addition of Metachromatic Leukodystrophy to the Recommended Uniform Screening**

##### **Panel**

**AGENCY:** Health Resources and Services Administration (HRSA), Department of Health and Human Services.

**ACTION:** Notice.

**SUMMARY:** The Health Resources and Services Administration (HRSA) published a Federal Register notice on August 14, 2025 (90 FR 39196), requesting comments from the public on the potential recommendation of adding Metachromatic Leukodystrophy (MLD) to the Recommended Uniform Screening Panel (RUSP). After consideration of public comments and evidence-based reports, HRSA recommended to the Secretary of Health and Human Services (HHS) that MLD be added to the RUSP. The Secretary of HHS has accepted the recommendation as detailed in this notice. Conditions listed on the RUSP are part of the evidence-informed preventive health guidelines supported by HRSA for infants, children, and adolescents. Non-grandfathered group health plans and group health insurance issuers are required to cover screenings included in these HRSA-supported comprehensive guidelines without cost-sharing (e.g., copayment, co-insurance, etc.). Please see the RUSP (<https://newbornscreening.hrsa.gov/about-newborn-screening/recommended-uniform-screening-panel>) for additional information.

**FOR FURTHER INFORMATION CONTACT:** CDR Leticia Manning, Newborn Screening Team Lead, Division of Services for Children with Special Health Needs, Maternal and Child Health Bureau, HRSA, 5600 Fishers Lane, Rockville, Maryland 20857 or [NBSPrograms@hrsa.gov](mailto:NBSPrograms@hrsa.gov).

**SUPPLEMENTARY INFORMATION:** The RUSP is a list of conditions that the Secretary of HHS recommends for states to screen as part of their state universal newborn screening (NBS) programs. Conditions on the RUSP are chosen based on evidence that supports the potential net benefit of screening, the ability of states to screen for the disorder, and the availability of effective treatments. Although states ultimately determine what conditions their NBS program will screen for, it is recommended that every newborn be screened for all conditions on the RUSP. Conditions listed on the RUSP are part of the comprehensive preventive health guidelines supported by HRSA for infants, children, and adolescents under section 2713 of the Public Health Service Act. Non-grandfathered group health plans and health insurance issuers are required to cover screenings included in these HRSA-supported comprehensive guidelines without charging a co-payment, co-insurance, or deductible for plan years beginning on or after the date that is one year from the Secretary's adoption of the condition for screening.

The Advisory Committee on Heritable Disorders in Newborns and Children (ACHDNC), now inactive, was tasked with reviewing available scientific evidence and then making recommendations to the Secretary of HHS regarding what conditions should be on the RUSP. When a condition is nominated, ACHDNC determines whether there is sufficient evidence available for early screening and refers it to ACHDNC's Evidence Review Group (ERG). The ERG is responsible for identifying and assessing all available evidence and summarizing for ACHDNC the strength and effectiveness of the evidence found on the net benefit of screening, the ability of states to screen for the condition, and the availability of effective treatments. The ERG completed an evidence review for MLD. Following the completion of the evidence review for MLD, but prior to issuing a recommendation to the Secretary on the inclusion of MLD to the RUSP, the ACHDNC was terminated.

The condition for addition, MLD, is a rare genetic condition that leads to progressive motor and brain damage. Children with early-onset forms of the condition who do not receive treatment before symptom onset experience motor function loss/paralysis and neurological

impairment followed by death at 5-6 years of age. Gene therapy is extremely effective if started early in life, with those who were placed on the earliest treatment pilots continuing to live into their mid-teen years today.

### **Summary of Public Comments:**

A Federal Register notice sought public comment on the potential recommendation of including or not including MLD on the RUSP. HRSA requested that the respondents consider the ERG's report summary on MLD and the suitability of state NBS programs screening for MLD within the newborn period in their response. HRSA considered all public comments as part of its deliberative process along with review of the completed MLD evidence review report prior to making a recommendation to the Secretary of HHS. A total of 98 respondents commented on the inclusion of MLD on the RUSP. Of these, 96 responses (98 percent) expressed support to add MLD to the RUSP, 1 response (1 percent) opposed its addition, and 1 response (1 percent) provided feedback on awaiting results from pilot studies and early adopting states to inform implementation. The responses in support of or against adding MLD to the RUSP are summarized below.

#### *Comments on adding MLD to the RUSP*

The majority of respondents (96 responses or 98 percent) described benefits of adding MLD to the RUSP including highly accurate screening for MLD with minimal false positives, if any; an FDA-approved gene therapy proven to prevent symptoms if administered before onset of disease; and early diagnosis can save lives of children with MLD.

The respondent that opposed adding MLD to the RUSP cited resource challenges for implementing MLD screening in public health laboratories and the high cost of treatment as reasons why MLD should not be added to the RUSP. However, HRSA notes that adding a condition to the RUSP does not require states to implement screening immediately. States

determine their resource allocations for NBS screening based on their specific state budget and public health priorities.

After consideration of the evidence review report and public comments, no changes were made to the recommendation and HRSA recommended to the Secretary of HHS that MLD be included for addition to the RUSP.

### **Acceptance of Recommendation**

On December 16, 2025, the HHS Secretary accepted HRSA's recommendation. The RUSP is updated and can be accessed at the following link:

<https://mchb.hrsa.gov/programs/newborn-screening>.

**Thomas J. Engels,**

*Administrator.*

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