

# **Newborn Screening for Krabbe Disease Expedited Review: Phase 1 Update**

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# Evidence Review Group (ERG)

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# Technical Expert Panel Members

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# Previous Recommendation

# ACHDNC Recommendation

- In February 2023, the ACHDNC voted against recommending to the Secretary, HHS, that Krabbe disease should be added to the RUSP following a tied vote.
- Summary from the Chair from March 2023 highlighting opportunities to weigh net benefits
  - Additional information about the efficacy of HSCT for early infantile Krabbe disease
  - Evidence regarding harms of HSCT in the first 2-months after birth
  - Additional information on outcomes for infants who are at risk of late infantile Krabbe disease

# Expedited Review

- In May 2023, the ACHDNC approved an expedited review based on an updated nomination.
- This is the first expedited review.

# Infantile Krabbe Disease

- Onset before 12 months
- Reduced galactocerebrosidase (GALC) activity
- Psychosine  $\geq 10$  nM in a dried blood spot
- Detection through a similar screening approach
  - GA, IL, IN, KY, MO, NY, PA, SC, TN
- Use of unambiguous terminology will be critical.

# Steps in an Expedited Review



# Updated Evidence Review

- Focus on infantile Krabbe disease as nominated (low GALC and psychosine  $\geq 10$  nM in the dried-blood spot)
- Bridge search of published data
- Grey literature
  - Pending: standardized assessment of development of subjects with Krabbe disease identified through newborn screening and treated with HSCT in early infancy
- Updated survey of states that use psychosine as a second-tier test
  - Cases identified and positive predictive value
  - Any outcomes that might be available. The nomination letter identifies 11 cases
- Updated decision analytic modeling reflecting the new target of screening

# Questions