

Approach to Prioritizing Nominations for the Recommended Uniform Screening Panel

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Background

- Potential increase in nominated conditions
 - Newborn screening technology advancements (e.g., additional conditions that could be multiplexed together in screening; genetic sequencing)
 - Treatment advances (e.g., gene therapy; novel targeted therapies)
- Concerns about limited capacity to meet demands of potential increase in the number of nominated conditions

Current Pace

CONDITION	Date Submitted	Evidence Review Referral	RUSP Recommendation
Krabbe Disease (2 nd)	7/21	Approved 05/22	Pending
GAMT Deficiency (3 rd)	6/21	Approved 08/21	Approved 05/22
MPS II	12/20	Approved 05/21	Approved 02/22
Cerebrotendinous Xanthomatosis	8/18	NOT Approved 11/18	
SMA (2 nd)	2/17	Approved 05/17	Approved 02/18
GAMT (2 nd)	8/16	NOT Approved 11/16	
GAMT (1 st)	11/15	NOT Approved 05/16	
ALD (2 nd)	09/13	Approved 01/14	Approved 08/15
ALD (1 st)	02/12	NOT Approved 09/12	
MPS I	02/12	Approved 05/12	Approved 02/15
Pompe Disease (2 nd)	02/12	Approved 05/12	Approved 05/13
22q11 Deletion Syndrome	09/11	NOT Approved 01/12	
CCHD	10/09	Approved 01/10	Approved 09/10
Hyperbilirubinemia	07/09	Approved 01/10	NOT Approved 01/12
Hemoglobin H Disease	04/09	Approved 09/09	NOT Approved 05/10
SMA (1 st)	06/08	NOT Approved 11/08	
Niemann-Pick Disease	01/08	NOT Approved 10/08	
Krabbe Disease (1 st)	12/07	Approved 08/08	NOT Approved 09/09
Fabry Disease	12/07	NOT Approved 08/08	
Pompe Disease (1 st)	10/07	Approved 01/08	NOT Approved 10/08
SCID	09/07	Approved 01/08	Approved 02/09

Previous ACHDNC Activity

- February 2022 Committee Meeting – Initial discussion about the capacity to review conditions

Background

- The Nomination & Prioritization (N&P) Workgroup has criteria to review submitted nomination packages
- The N&P Workgroup has a finite capacity
- The ACHDNC has restrictions on the number of reviews that could be considered at any given time
- No criteria have been defined for prioritization of multiple simultaneously nominated conditions

THIS HAS NOT BEEN A CONCERN YET

Next steps

Form a Workgroup with Committee members (past and present) to develop criteria and a process for prioritizing the review of nomination packages, with input from stakeholders

Prioritization is Common

- US Preventive Services Task Force approach
 - Nominated topics are reviewed to determine if they are in scope and a new topic. If in scope and a new topic, then it enters a process for prioritization
 - A request for feedback on all active and potentially new topics is sent to Task Force members and partner organizations. Asked to vote on whether the condition is high-, moderate-, or low-priority for review in the next 12 to 18 months
 - Topic Prioritization Workgroup then assigns a tentative priority category
 - The Task Force votes

Key Points

- Prioritization is about cadence; it cannot be used to stop a condition from moving forward to evidence review if recommended by the usual Nomination and Prioritization Workgroup methods.
- When prioritization is needed, the process should be transparent to all stakeholders.

Key Principles for Prioritization

- Maximize public health benefit, considering
 - Prevalence
 - Expectation of benefit
 - Potential harms
 - Screening test validity
 - Reduction of inequities
 - Ability to implement comprehensive screening
 - Balanced portfolio
- Approach could range from a qualitative assessment to a more formal point system

Additional Benefits of the Prioritization Process

- Could be used to help further structure and provide clarity about the nomination process, including what is needed from nominators

Potential Process

- In the event there must be prioritization, the Nomination and Prioritization workgroup will make recommendations to the ACHDNC
- The Nomination and Prioritization will regularly present the list of conditions that have been nominated but not yet been prioritized for review

Questions