

# Summary of the Revised ACHDNC Condition Nomination Process

August 8, 2024

# Background

- Nominations for conditions to be considered to the RUSP are critical to the work of the ACHDNC
- Challenges
  - Nominators feel burden to make a compelling and comprehensive case for adding a condition to the RUSP
    - Exert a tremendous amount of time and effort
  - Jargon on the nomination form
- Goal
  - Simplify the process for nominators while maintaining the central role that nominations have for the evidence review and the ACHDNC recommendation process

# Nomination Revision Process

- Feedback from groups of 5 recent/current nominations
- Discussion at previous ACHDNC meetings
  - Small-group listening sessions (Nov 2023)
  - Large-group discussion (Jan 2024)
- Input from ACHDNC standing workgroups
  - Follow up and Treatment
  - Education and Training
  - Laboratory standards and Procedures
- Public comment received in response to Federal Register Request For Information from March - April 2024



# Step 1: Pre - Nomination

- Condition nomination group complete preliminary nomination form
- The [Preliminary Nomination Form](#) consists of the following questions:
  1. Is there a newborn screening test available?
  2. Is there agreement about the case definition of the targeted condition and diagnostic confirmation after a positive newborn screen?
  3. Is there a prospective population-based newborn screening project that has identified at least one infant with the condition?
  4. Can identification of the targeted condition before clinical presentation allows provision of effective therapy and improve outcomes for screened infants?
- If yes to each question, nominators submit 1-3 peer-reviewed references for each question with a brief explanation.
- Helps the ACHDNC understand if there is enough evidence to move to a full evidence review, but does not replace a full review

# Review of Step 1

- The Nomination and Prioritization (N&P) Workgroup is selected by the Committee Chair. The workgroup consists of selected Committee members.
- The N&P Workgroup reviews the preliminary nomination form and verifies that the nomination meets the four basic requirements needed for a condition to be considered for review.
- The Designated Federal Official (DFO) communicates the N&P's findings to the Nominators.
  - Nominators are encouraged to meet with the Committee Chair and DFO to discuss next steps (which may or may not include the completion of the full nomination package).

# Step 2: Full Nomination Package



Once the preliminary nomination form is verified by the N&P Workgroup. The nominators may complete the full nomination package.

## **Nomination Package Sections**

- The Condition
- Screening
- Impact of Screening
- Other considerations
  - Any information not captured in the previous categories
- References

# Review of Step 2

- The Nomination and Prioritization (N&P) Workgroup reviews the full nomination package and creates a summary for the Committee's assessment.
- The assessment is shared with the Committee at a Committee meeting.
- The Committee votes on whether or not to move the condition forward to full evidence review.

# ACHDNC Criteria to consider whether to proceed with a full evidence-based review

1. Is the nominated condition(s) *medically serious*?
2. Is the *case definition* and the spectrum of the condition(s) well described, to help predict the phenotypic range of those children who will be identified based on population-based screening.
3. Are *prospective pilot data* from population-based assessments available for this condition?
4. Does the screening test(s) have established *analytic validity*?
5. Are the *characteristics of the screening test(s)* reasonable for the newborn screening system (among other aspects, a low rate of false positives)?
6. Is there a widely available *confirmatory test/diagnostic* process, with CLIA and/or FDA approval as appropriate ?
7. Are there defined *treatment* protocols for the condition when identified?
8. Do the results have *clinical utility*, balancing benefits and harms?
9. Will screening identify those most likely to benefit from treatment?



# Discussion