

# Update on the Revised ACHDNC Condition Nomination Process

May 10, 2024

# Background

- Nominations for conditions to be considered to the RUSP are critical to the work of the ACHDNC
- Challenges
  - Pressure on nominators to make a compelling and comprehensive case for adding a condition to the RUSP leads to a tremendous amount of 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

# 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

# Simplified Approach

- Preliminary nomination to be assessed by the Nomination and Prioritization Workgroup addressing four questions whether:
  1. Availability of a newborn screening test
  2. Agreement about case definition and diagnostic confirmation after a positive screen
  3. There is a prospective population-based newborn screening project that has identified at least one infant with the condition
  4. Identification before clinical presentation allows provision of effective therapy to improve the outcomes for the infant screened
- If yes to each, nominators submit 1-3 peer-reviewed publications for each question and partners with HRSA, ACHDNC Chair, and selected ACHDNC members to assemble a complete nomination package
- Helps the ACHDNC understand if there is enough evidence to move to a full evidence review, but does not replace a full review

# Advantages

- The preliminary nomination should require much less work and provide the foundation for having the ACHDNC provide guidance and support about what information is needed for the complete nomination package
- The complete nomination will allow advocates to focus on key factors for moving to evidence review without needing to replicate what will eventually be done by the evidence review group

# Next Steps

- ACHDNC approval of the revised approach
- Finalization of new nomination forms – preliminary and complete
- Development of additional resources, including a glossary of key terms

# Discussion