

Reviewing the ACHDNC Evidence-review Process: Nomination Process

Presented to the Advisory Committee on Heritable Disorders in
Newborns and Children

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Objectives

- Inform the ACHDNC about ways to strengthen the decision-making process and develop a future manual of procedures

Approach and Timeline through May 2021

Date	Review of the Committee Process Discussions
Feb 2019	Expert Advisory Panel Meeting to review the process
April 2019	Systematic Evidence Review and Population-level Health
July 2019	Public Health Impact Assessment
<i>Oct 2019 – April 2020</i>	<i>Legislative Hiatus</i>
May 2020	Recap of progress
	Considerations regarding values assessment and using this in the recommendation process
August 2020	Assessing values – recommendations, decision-making criteria/matrix/recommendations
December 2020	Decision-making and decision matrix
February 2021	Evaluating conditions on the RUSP
	Nomination Process/Form
May 2021	Overview of process review Summary of recommendations for the future

The Review Process: Four Focus Areas

1. Nomination

2. Review Process

- *Add on: Assessing Values in the decision-making process*

3. Decision Matrix

4. Review of Current Conditions on the RUSP

Guidance on Nominating a Condition on the Committee's website

<https://www.hrsa.gov/advisory-committees/heritable-disorders/rusp/nominate.html>

Nominate a Condition

Conditions for consideration by the Committee for the [Recommended Uniform Screening Panel \(RUSP\)](#) (PDF - 190 KB) must be nominated.

The Committee encourages individuals and organizations to form multi-disciplinary teams to submit nominations for conditions to be considered for inclusion on the RUSP. Teams should include researchers and/or clinicians with expertise on the condition being nominated, advocacy and/or professional organizations with knowledge of issues relevant to newborn screening, and interested consumers/individuals.

To apply, the lead nominator or proponent should submit a nomination package.

Nomination Package Components

- Cover letter by the lead nominator that identifies all multi-disciplinary team members and their organizational affiliation(s), if applicable;
- Letters of support (from multi-disciplinary team members), if applicable;
- Completed [Conflict of Interest Disclosure Forms](#) (PDF - 63 KB)* from all team members;
- Responses to [Nomination Form](#) (PDF - 247 KB) and;
- Supporting data and scientific/clinical references to substantiate all responses to Nomination Form questions.

*A potential or actual conflict of interest exists when commitments and obligations are likely to be compromised by the nominator(s)'s other material interests or relationships (especially economic), particularly if those interests or commitments are not disclosed.

Next Steps

Nomination and Prioritization Workgroup

The Committee's [Nomination and Prioritization \(N&P\) Workgroup](#) reviews the completed Nomination Package and compiles a summary for Committee consideration. The Committee decides if sufficient evidence is available, and votes to assign, or not assign, the nominated condition to the external Condition Review Workgroup. Nominators whose conditions are not assigned to the Condition Review Workgroup are provided with feedback.

Condition Review Workgroup

The external [Condition Review Workgroup](#) completes a systematic evidence-based review, provides updates, and presents a final report to the Committee on assigned conditions.

Committee Deliberations and Vote

The Committee discusses and deliberates on the evidence presented by the Condition Review Workgroup. The Committee uses a [decision matrix](#) (PDF - 255 KB) to guide their final decisions. Then the Committee votes to recommend or not recommend adding the nominated condition to the RUSP for consideration by the Secretary of Health and Human Services. Nominators whose conditions are not recommended for addition to the RUSP are provided with feedback.

Final Decision

The [Secretary of Health and Human Services](#) makes the final decision on whether to add, or not add, a recommended condition to the RUSP.

The Nomination Process

- Nominators prepare Nomination Package and submit to HRSA
- HRSA reviews nomination package for completeness
- Committee's Nomination and Prioritization (N&P) Workgroup reviews nomination for 6 key questions:
 - *Condition Seriousness.*
 - *Case Definition*
 - *Analytic validity*
 - *Clinical utility*
 - *Available Treatments*
 - *Prospective Pilot Data*
- N&P Workgroup assesses whether all requirements are met and there is sufficient evidence to present to the full Committee for consideration of sending the nomination to evidence review
- If requested, the NBS Evidence Review Group begins the full evidence review, to be completed within 9 months

Review of The Nomination Process

- Guided by the needs of the Evidence Review and Committee decision-making
- How can the Nomination Process align to effectively facilitate the evidence review or decision-making?
- Review information requested on the Nomination Form
 - Additional information
 - Timing of information or evidence (what information is better to have early in the process)
- Ensure that any changes to the nomination and review process are described to potential nominators.

Nomination Form – Suggested edits/additions

Current Nomination Form	Selected areas of expansion
Nominator contact information	<ul style="list-style-type: none">• Other experts in screening or treatment of the condition
Condition information and treatment	<ul style="list-style-type: none">• Specific case definition, screening target
Evidence-based information - screening <ul style="list-style-type: none">✓ Validity of laboratory screening test✓ Availability of accurate confirmatory and diagnostic testing✓ Prospective population-based pilot study	<ul style="list-style-type: none">• Pilot study contacts• Screening algorithm piloted and results• Confirmatory, STFU, and LTFU specialists• Available long-term follow-up and plans for collecting long-term outcomes
Key references from scientific publications	<ul style="list-style-type: none">• Available registries• Availability of unpublished data

Questions for the Advisory Committee

- Do the elements on the nomination form align with what will be needed to recommend evidence review?
 - If not, what should be added and how would it be used?
- What opportunities are available to facilitate the nomination process?