ACHDNC Decision Matrix Tool

August 8, 2024

ACHDNC
Secretary's Advisory Committee on Heritable Disorders in Newborns and Children

Purpose of the matrix tool

- The tool is meant to support decision making
- It is not meant to make the decision

ACHDNC Decision Matrix (draft)

	Magnitude of Net Benefit		
Certainty of Net Benefit	Substantial	Moderate	Zero, Small or Negative
High	A	B	C
Moderate	B	B	C
Low	I (insufficient)		

Letter Grade	Description	Action
A	High certainty of substantial net benefit	Recommend addition to the RUSP
B	At least moderate certainty of at least moderate net benefit	Discuss and vote on recommending addition to the RUSP
C	At least moderate certainty of less than moderate net benefit	Do not recommend addition to the RUSP; Identify evidence gaps
I	Low certainty of net benefit	Do not recommended addition to the RUSP; Identify evidence gaps

Public Health Impact assessment for implementation in 2 years:	
# of States reporting effort required as low	
# of States reporting effort required as moderate	
# of States reporting effort required as high	

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Decisions for designations

- Conditions with an “A” designation will be forwarded to the Secretary with a recommendation to add to the RUSP
- Conditions with a “B” designation MAY be forwarded to the Secretary with a recommendation to add to the RUSP after discussion and a separate vote
- Conditions with a “C” designation will not be forwarded to the Secretary, but evidence gaps will be identified and shared with nominators
- Conditions with an “I” designation will be not be forwarded to the Secretary, but evidence gaps will be identified and shared with nominators

Process for B designation and action

- Based on assessment of the magnitude of net benefit, and the certainty of net benefit, the committee votes to assign a B designation
- Based on additional discussion of the evidence and an assessment of the anticipated impact of adding the condition in terms of individual, family and public health benefit, the committee votes on whether to recommend adding the condition to the RUSP
- This separates agreement on the evidence from agreement that the condition should be added to the RUSP

Implementation issues

- The matrix requires judgements in two areas: certainty of net benefit and magnitude of net benefit
  - Certainty of net benefit: there are well-established approaches for this decision
  - Magnitude of net benefit: this is more complex, especially in the setting of creating new levels of net benefit, “substantial” and “moderate”

Public health impact assessment

Assessment process overview

- The AC initiates the PHIA process when AC votes to move to the Evidence Review Group
- Pilot states are surveyed and results are distributed to all other states
- Survey represents the diversity of state population size and overall newborn screening resources

Pilot state survey

Survey items

- Will use current ERG/APHL methods to survey pilot states
- Includes questions regarding screening testing (first and higher tier), confirmatory testing, diagnosis and first year treatment

Survey items

- For each area, questions will cover whether and what new equipment/ staff/medical expertise was required
- Will request estimates of costs involved, focusing on reasonable ranges

Cost estimates caveats

- Recognize that the most important metric is ease of implementation, which isn't captured by cost estimates
- It will be useful for other states contemplating screening implementation to know what they will need rather than how much it will cost; what access exists to treatments, and how it worked in other states
- Still, cost estimates and opportunity costs are likely to provide useful information as the required level effort is estimated

Example Pilot test report

First tier testing
- Equipment:
- Staff:
- Expertise:

Higher tier testing:
- Sent out, or
- Performed in house
  - Equipment:
  - Staff:
  - Expertise:

Follow up—diagnosis:
- Expertise
- Availability

Follow up—treatment:
- Expertise
- Availability

Cost estimates for implementation (ranges):

Caveats for cost estimates including comments on opportunity costs:

Pilot results (total tests, positive tests (first tier/higher tier), confirmed cases:

Issues in implementation:

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States survey

Based on pilot state information

1. If the condition is added to the RUSP, what resources or additional support would you need to implement within 2 years (e.g., external support for start up, regionalization agreements, other)?
2. If you could not implement within 2 years, what would be the barriers (could include competing priorities such as implementing other RUSP additions, other state laboratory priorities, funding and staffing challenges, etc.)?

3. What is your estimate of the effort required for implementation within 2 years?

- Based on comparison with other RUSP additions:
  - We estimate the required effort will be **LOW** (e.g., minimal costs, little or no new equipment/staff/expertise, treatment readily available)
  - We estimate the effort will be **MODERATE** (e.g., significant costs, new expertise for testing, new equipment, new resources for referrals)
  - We estimate the effort will be **HIGH** (e.g., substantial new investment necessary; required staff, expertise, and/or referral resources unlikely to be available within 2-3 years)

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Discussion