Duke Clinical Research Institute

Users' Guide to the SACHDNC Decision Matrix

Alex R. Kemper, MD, MPH, MS

January 31, 2013

Background

- In September 2012, the SACHDNC approved the use of a decision matrix to assist with the development of recommendations regarding conditions nominated to the RUSP.
- Based on a 2-step process involving assessment of
  - *Net Benefit: Benefits - Harms*
  - *Capability of state newborn screening programs to adopt comprehensive screening*

Net Benefit

- A – high certainty of significant net benefit
- B – moderate certainty of significant net benefit
- C – high or moderate certainty of a small to zero net benefit
- D – high or moderate certainty of a negative net benefit
- L – low certainty

Assigning Net Benefit (Benefits – Harms)

- Most important consideration is to the child being screened
- Considerations
  - *The overall public health burden (birth prevalence, severity)*
  - *Benefits of early detection and treatment to affected children*
  - *Harms related to screening, diagnosis, and treatment, both to affected and unaffected children*
- False-positive screens are an important harm. However, the impact of false-positive screens can vary based on condition
- Compelling evidence needed to justify screening for late-onset disease
- The SACHDNC does not use a single defined metric for classifying net benefit

Capability to Screen

- 1 – high to moderate feasibility, most ready to begin
- 2 – high to moderate feasibility, most have developmental readiness
- 3 – high to moderate feasibility, most unprepared
- 4 – low feasibility

Assigning the Capability to Screen

- Technical and clinical feasibility is central
- Overemphasis of readiness could delay adoption.
- Assessment of readiness can
  - *help identify needs that can be addressed*
  - *guide implementation activities*

Assigning the Capability to Screen

- Technical and Clinical Feasibility
  - *An established screening test*
  - *A clear approach to diagnostic confirmation*
  - *Accepted treatment*
  - *Plan for long-term follow-up*
- Readiness
  - *Availability of resources for screening; diagnostic confirmation; long-term follow-up, including treatment*
  - *Authorization for screening*

Examples

- SCID
  - *"A" net benefit, "4" →"2" ability to screen*
- CCHD
  - *"A" net benefit, "4" →"3" →"2" ability to screen*
- Pompe disease
  - *2006 – "B" net benefit, "4" ability to screen*
- Hemoglobin H Disease
  - *"L" net benefit, ? ability to screen*
- Hyperbilirubinemia
  - *"C" net benefit, ? ability to screen*
- Krabbe Disease
  - *"L" net benefit, ? ability to screen*

Lessons Learned

- Conditions rated as B or L should include specific guidance about what future research is needed
- A score for the ability to screen does not need to be assigned if the condition is unlikely to be recommended for screening based on net benefit
- There is overlap between readiness and feasibility. These terms are not meant to be mutually exclusive, but provide a framework to assure all issues are considered

Questions / Comments