



Challenges with conducting NBS pilot studies



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DACHDNC Process

- Recommendations about inclusion of conditions on the RUSP are evidence-based
- Evidence in this context requires quality studies addressing
 - Test characteristics
 - Natural history/spectrum of the condition
 - Impacts of early detection and intervention on child morbidity and mortality

Challenges

- Conduct of research in this public health domain often requires a collaboration with state departments of health
- Many state departments of health
 - Do not consider research to be part of their mission
 - Are prohibited from conducting certain kinds of research
- Variability by IRBs in expectations for human subject protections

Experience

- A number of states have effectively collaborated with investigators to conduct valuable NBS research
- A number of states have been unable to support a number of valuable projects
- Barriers to research invite implementation of new tests through state mandates rather than an evidence review process

Proposal for development

- DACHDNC discussion and potential recommendations for a national infrastructure to conduct population-based screening on a research basis
 - Research conducted through state-based programs in order to mirror potential future applications in state-based NBS systems
- More detailed presentation at our next meeting