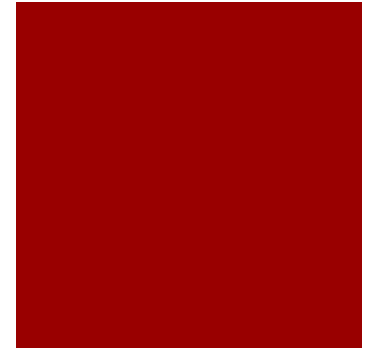


DACHDNC Work Group on Pilot Studies

Jeffrey Botkin, Chair

Work Group Members



- Jeff Botkin
- Dietrich Matern
- Michael Watson
- Anne Comeau
- Mark McCann
- Scott Shone
- Kellie Kelm
- Carla Cuthbert
- Melissa Parisi
- Tiina Urv
- Debi Sarka

Charge to the Work Group



- Recognize and support current efforts regarding pilot studies and evaluation
- Identify other resources that could support pilot studies and evaluation
- Identify the information required by the Committee to move a nominated condition into the evidence review process (i.e., define the minimum pilot study data required for a condition to be accepted for evidence review)

Events



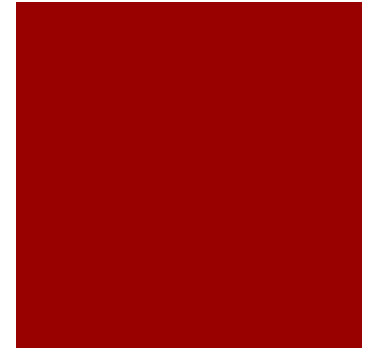
- *Newborn Screening Saves Lives Reauthorization Act of 2014 (Public Law No: 113-240)*
 - **TEXT OF SEC. 12. INFORMED CONSENT FOR NEWBORN SCREENING RESEARCH.**
 - *(a) IN GENERAL.—Research on newborn dried blood spots shall be considered research carried out on human subjects meeting the definition of section 46.102(f)(2) of title 45, Code of Federal Regulations, for purposes of Federally funded research conducted pursuant to the Public Health Service Act until such time as updates to the Federal Policy for the Protection of Human Subjects (the Common Rule) are promulgated pursuant to subsection (c). For purposes of this subsection, sections 46.116(c) and 46.116(d) of title 45, Code of Federal Regulations, shall not apply.*
 - *(b) EFFECTIVE DATE.—Subsection (a) shall apply only to newborn dried blood spots used for purposes of Federally funded research that were collected not earlier than 90 days after the date of enactment of this Act.*

Opportunities



- *ProSpective Evaluation of NewbOrn InfanIs with Spinal Muscular Atrophy Identified via Newborn Screening: SPOT SMA
 - Kathryn Swoboda, PI
 - NIH/NICHD funded*
- A specific aim of the study was to assess public attitudes on parental permission for pilot studies for SMA
- Discussions about the feasibility of expanding this SA to address attitudes of other stakeholders

Panel Discussion



- **CDC Activities**
 - Carla Cuthbert
- **NIH/ACMG Activities**
 - Michael Watson
 - Tiina Urv
- **State Health Department Activities**
 - Anne Comeau