



Discretionary Advisory Committee on Heritable Disorders in Newborns and Children

Pilot Study Workgroup Update



Members

- Jeff Botkin – Chair
- Carla Cuthbert
- Anne Comeau
- Mike Watson
- Mark McCann
- Melissa Parisi
- Scott Shone
- Tiina Urv
- Kelly Kelm
- Dieter Matern

Goals and Tasks

- Recognize and support current efforts regarding pilot studies and evaluation
- Identify other resources that could support pilot studies and evaluation
- Provide recommendations to the Secretary that will further support the DACHDNC condition nomination process.
- Consider ways on how to create a network of states that could support infrastructure of doing pilot studies.
- 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)

First call

- Brief reports from Mike Watson for the NBSTRN, Tiina Urv for NIH, Carla Cuthbert for CDC, Ann Comeau from MA
- Identified other experts who might be included on the workgroup permanently and on an ad hoc basis.

Next Steps

- DACHDNC January Meeting – Panel presentation
 - CDC, NIH, NBSTRN, and potentially others to inform the Committee on pilot programs
- Look back at previous condition nominations and analyze the pilot studies that were included
- Next call in October
- Create a name for the workgroup