

Follow Up and Treatment Workgroup Update

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Advisory Committee on Heritable Disorders in Newborns and Children

2019 Follow-up and Treatment Workgroup

ACHDNC MEMBERS

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- Kamila B. Mistry, PhD, MPH
- Annamarie Saarinen

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Society for Inherited Metabolic Disorders
- Christopher A. Kus, MD, MPH (FUTR Co-Chair)
Association of State & Territorial Health Officials
- **Jennifer M. Kwon, MD, MPH, FAAN**
Child Neurology Society
- Robert J. Ostrander, MD
American Academy of Family Practice Physicians
- Jed L. Miller, MD, MPH
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WORKGROUP MEMBERS

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- Tracey Bishop
- Amy Brower, PhD
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- Margie A. Ream, MD, PhD
- Elna Saah, MD
- Joseph H. Schneider, MD, MBA, FAAP
- Marci Sontag, PhD
- Janet Thomas, MD

Workgroup Discussion

Gaps, topics or issues, specific projects?

- Continued emphasis on long-term follow-up (LTFU). When a new condition is introduced, we should be thinking about LTFU from the beginning.
- Lack of a national network that can coordinate care and collect data in a standardized way.
- Engaging the EMR and AI industry is a current gap that could support more efficient data collection initiatives.
- Defining who is responsible for LTFU and at what stages.
- Financial resources for LTFU is a major gap/ federal – state partnership.
- We need to learn about access to care after diagnosis and describing the barriers.

Gaps, topics or issues, specific projects

- There is a need to strengthen LTFU standardization by developing core outcomes or a minimum data set.
- Children who are identified with a condition outside of NBS and enter LTFU?
- Communicating the importance of LTFU to families.
- The Advisory Committee on Heritable Disorders in Newborns and Children – not all heritable disorders are NBS-related.

Feedback on the components of the RUSP condition nomination and evidence review

- The nomination process should consider a “blueprint” for LTFU that includes both patient follow-up and measuring health outcomes.
- It may be important to include a description of the potential barriers to care.
- It is important that the process is able to discern the merit of the information and take into consideration the resources that were available to the condition in the nomination process.