

Follow-up and Treatment Workgroup 5/9/2016

Stephen McDonough MD
Old Pediatrician

HRSA Office Building 5/9/2016

- 19 present at meeting
- 8 participating by phone
- Consensus to work on two priorities
- Clinical quality measures
- Medical formulas and foods

Promoting the Role of Clinical Quality Measures to Promote LTFU of Newborn Screening

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Advisory Committee on Heritable Disorders in Newborns and Children
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Potential Roles for NewSTEPs in promoting CQM for NBS

- The focus of NewSTEPs is primarily on short term follow-up and the completion of the newborn screening process. We cannot change their mission, but we can leverage work they have already done to build CQM for timeliness of newborn screening and short term follow-up as well as to build networks of newborn screening programs.
- Existing case definitions will help define target populations for CQM.
- NewSTEPs could include lists of CQM for LTFU on their website along with educational materials about CQM as part of their technical support mission.
- Gathering initial values for CQM may be part of the process of transitioning from short term follow-up into long term follow-up and treatment.
- The end of short term follow-up is the beginning of long term follow-up making baseline LTFU CQM a potential starting point for future data capture and sharing.
- The primary role of NewSTEPs would be supporting communication.

Potential Roles for NBSTRN in promoting CQM for NBS

- The Newborn Screening Translational Research Network (NBSTRN) has created a Longitudinal Pediatric Data Resource (LPDR) that is a secure shared REDCap based newborn screening long term follow-up database with common and disease specific data elements.
- The considerable work that went into developing the LPDR, including public health considerations, provides a solid and valuable framework for CQM development and implementation.
- All CQM for LTFU of NBS should be based on fields already in the LPDR or that may need to be added.
- Codes and value sets for the LPDR should support CQM and guide requesting new codes and value sets.
- Data already stored in the LPDR could support testing and demonstrating new CQM for LTFU of NBS.
- The LPDR could integrate CQM and become a repository of CQM data subject to their ethical, legal, and social policies.

Three Proposed End Products for this Subcommittee Activity

1. A **Case Study** of successful use of clinical quality measures for follow-up of newborn screening - probably from cystic fibrosis or sickle cell disease.
2. A **Report to the Full Committee** highlighting the background, need, and opportunities to use clinical quality measures in long term follow-up of newborn screening. Appendices would suggest partners and stakeholders who could continue the work as well as potential need for new codes and value sets to support specific electronic quality measures. Potential on-going roles for NBSTRN LPDR, NewSTEPS, regional genetics collaboratives would also be included.
3. A **How to Guide for Developing Quality Measures** for newborn screening that could be distributed to newborn screening programs, regional genetics collaboratives, professional organizations, and disease specific organizations to help them begin the process of creating CQM.

Clinical quality measures

- Dr. Zuckerman will head a sub-workgroup on clinical quality measures and have at least one phone call between now and the August ACHDNC meeting.

Medical Foods

- White paper
- Draft of letter to be presented to ACHDNC to be sent to DHHS Secretary
- 11 people volunteer to work on this sub-workgroup
- Dr. Sue Berry has agreed to head the Medical Foods sub-workgroup
- Phone calls will occur between now and August ACHDNC meeting.