Follow-up and Treatment Workgroup Progress Report

ACHDNC Meeting, August 4, 2017
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Two Sub-workgroups (concluding)

• Medical Foods for Inborn Errors of Metabolism
  • Report affirmed by ACHDNC, final stages of editing
  • Publication planned (JAMA, Pediatrics, MM, GIM?)
• Quality Measures for Long-term Follow-up
  • Lead – Alan Zuckerman
  • Final report planned for November ACDNC meeting
    • Frame the report as “next step” in ACHDNC’s plan for improving long-term outcomes for children and adults with NBS conditions
    • Key work for the next 3 months
ACHDNC – Genetics in Medicine (2008)

Long-term follow-up after diagnosis resulting from newborn screening: Statement of the US Secretary of Health and Human Services’ Advisory Committee on Heritable Disorders and Genetic Diseases in Newborns and Children

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- Central components
  - Care coordination
  - Evidence-based treatment
  - Quality improvement

- Features
  - Quality chronic disease management
  - Condition-specific treatment
  - Care throughout lifespan
What questions should newborn screening long-term follow-up be able to answer? A statement of the US Secretary for Health and Human Services’ Advisory Committee on Heritable Disorders in Newborns and Children

Cynthia F. Hinton, PhD, MPH\textsuperscript{1}, Lisa Feuchtbaum, DrPH, MPH\textsuperscript{2}, Christopher A. Kus, MD, MPH\textsuperscript{3}, Alex R. Kemper, MD, MPH\textsuperscript{4}, Susan A. Berry, MD\textsuperscript{5}, Jill Levy-Fisch, BA\textsuperscript{6}, Julie Luedtke, BS\textsuperscript{7}, Celia Kaye, MD, PhD\textsuperscript{8}, and Coleen A. Boyle, PhD, MS\textsuperscript{9}
A framework for assessing outcomes from newborn screening: on the road to measuring its promise☆

Cynthia F. Hinton a,*, Charles J. Homer b, Alexis A. Thompson c, Andrea Williams d, Kathryn L. Hassell e, Lisa Feuchtbaum f, Susan A. Berry g, Anne Marie Comeau h, Bradford L. Therrell i, Amy Brower j, Katharine B. Harris k, Christine Brown l, Jana Monaco m, Robert J. Ostrander n, Alan E. Zuckerman o, Celia Kaye p, Denise Dougherty q, Carol Greene r, Nancy S. Green s, the Follow-up and Treatment Sub-committee of the Advisory Committee on Heritable Disorders in Newborns and Children (ACHDNC):
Framework for Assessing Outcomes from NBS

Hinton et al, 2016
Summary of “Quality Measures” Key Findings

• Quality measures are a crucial part of health and health care system
  • Improve outcomes (QI), “rapid-cycle” research, maintenance of certification
  • Quality is a critical component of “value-based care”

• Many different types of Quality Measures
  • Process - did something happen? (e.g. vaccine given)
  • Access to care - are patients able to get the care? (e.g. physician communicates well)
  • Health outcomes - hospitalizations, HGB A1C level, stroke
  • Quality of life - school attendance, perception of well-being

• Creating/collecting data for these measures can be challenging
  • NBS conditions are rare; difficult to gather evidence/demonstrate QMs = outcomes
  • State NBS programs do long-term follow-up in many ways; no national standard
  • LTFU of NBS unlikely to be included in CMS measure set(except SSD, EHDI)

• Different perspectives are needed to develop quality measures, especially the patient/family/consumer perspective
FUTR Workgroup Meeting

• 120 minutes of wide-ranging discussion, passion, ideas
• Time to assess next steps/future sub-workgroups
  • Quality measures are part of what we do next: “toolkit”
  • Alex Kemper/K.K. Lam will be presenting a “scan” of current LTFU activities across the U.S. at the November ACHDNC meeting
  • Other presentations planned at FUTR workgroup meeting in Nov.
• Strategy for organizing our efforts as we move forward
  • Children/adults with NBS conditions fit into 4 different populations
  • Each of the 4 populations offers opportunities for measuring and improving outcomes (many activities already happening)
  • Child/family perspective must be included in all 4 populations
FUTR Populations/Groups/Levels

1. Specific Conditions
   - Formal quality measures/QI activities, research networks
   - Opportunities for electronic medical record improvements
   - Family/Patient/Advocacy groups as critical driver of LTFU?
     - NORD, NBS Connect, others

2. All Conditions Identified by NBS
   - Typically state-level work to monitor and improve outcomes
   - Collaborative effort among states, APHL/NewSTEPS, NBSTRN (LPDR), NCC

3. CSHCN
   - National Survey of Children’s Health (NSCH) now includes CSHCN

4. All Children
   - Promote the use of outcomes relevant to CSHCN (including NBS conditions)
• Child health policy should reflect needs of CSHCN (including those with NBS conditions)
• CSHCN are especially vulnerable to the factors that affect the health of all children
• Policy that improves child health is especially beneficial to CSHCN