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

Alex R. Kemper, MD, MPH[1], Coleen A. Boyle, PhD[2], Javier Aceves, MD[3], Denise Dougherty, PhD[4], James Figge, MD, MBA[5], Jill L. Fisch[6], Alan R. Hinman, MD, MPH[7], Carol L. Greene, MD[8], Christopher A. Kus, MD, MPH[9], Julie Miller, BS[10], Derek Robertson, MBA, JD[11], Brad Therrell, PhD[12], Michele Lloyd-Puryear, MD, PhD[13], Peter C. van Dyck, MD, MPH[13], and R. Rodney Howell, MD[14]

- Central components
  - Care coordination
  - Evidence-based treatment
  - Quality improvement
- Features
  - Quality chronic disease management
  - Condition-specific treatment
  - Care throughout lifespan

ACHDNC – Genetics in Medicine (2011)

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[1], Lisa Feuchtbaum, DrPH, MPH[2], Christopher A. Kus, MD, MPH[3], Alex R. Kemper, MD, MPH[4], Susan A. Berry, MD[5], Jill Levy-Fisch, BA[6], Julie Luedtke, BS[7], Celia Kaye, MD, PhD[8], and Coleen A. Boyle, PhD, MS[1]

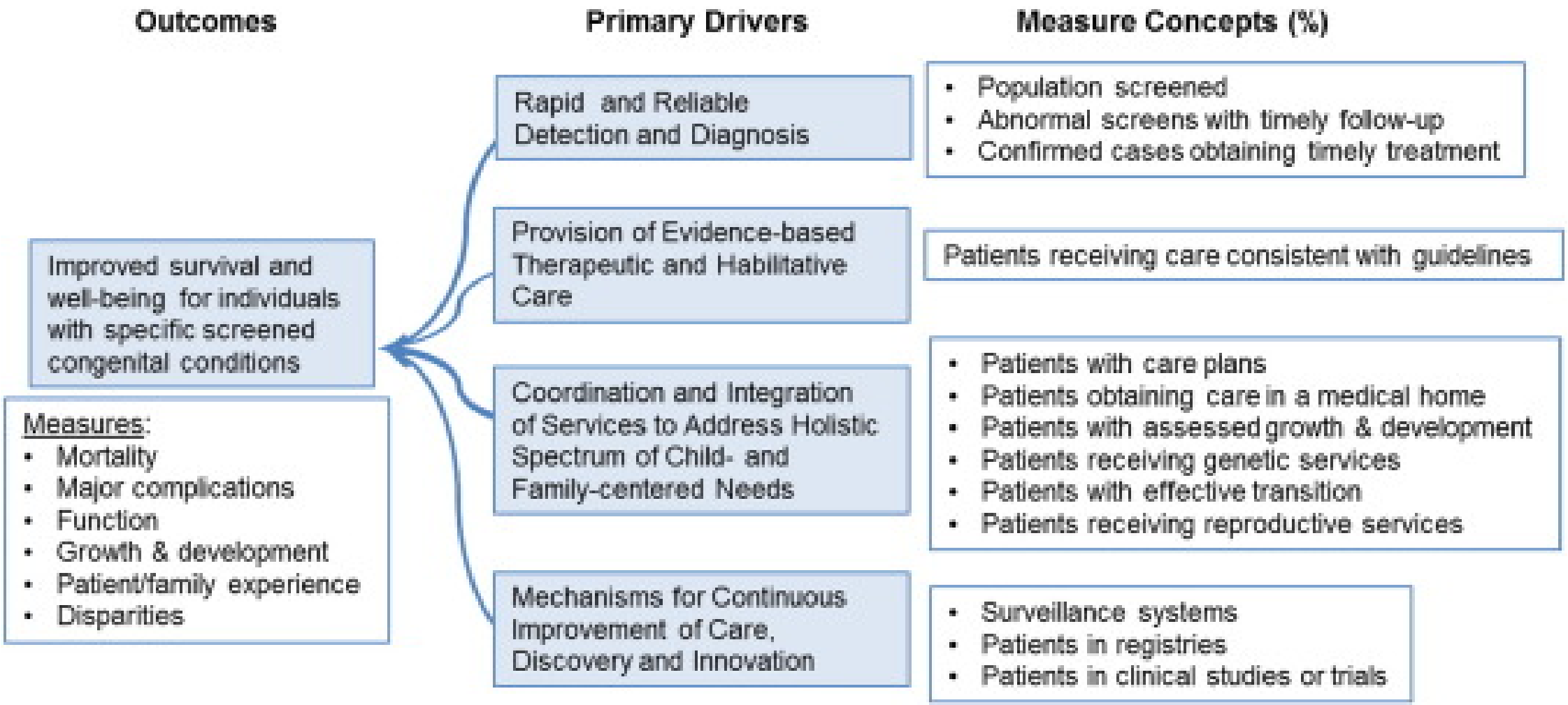
- Central components
  - Care coordination
  - Evidence-based treatment
  - Quality improvement
- Perspectives
  - State and nation
  - Primary/specialty providers
  - Families

ACHDNC – Molecular Gen & Metab (2016)

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”

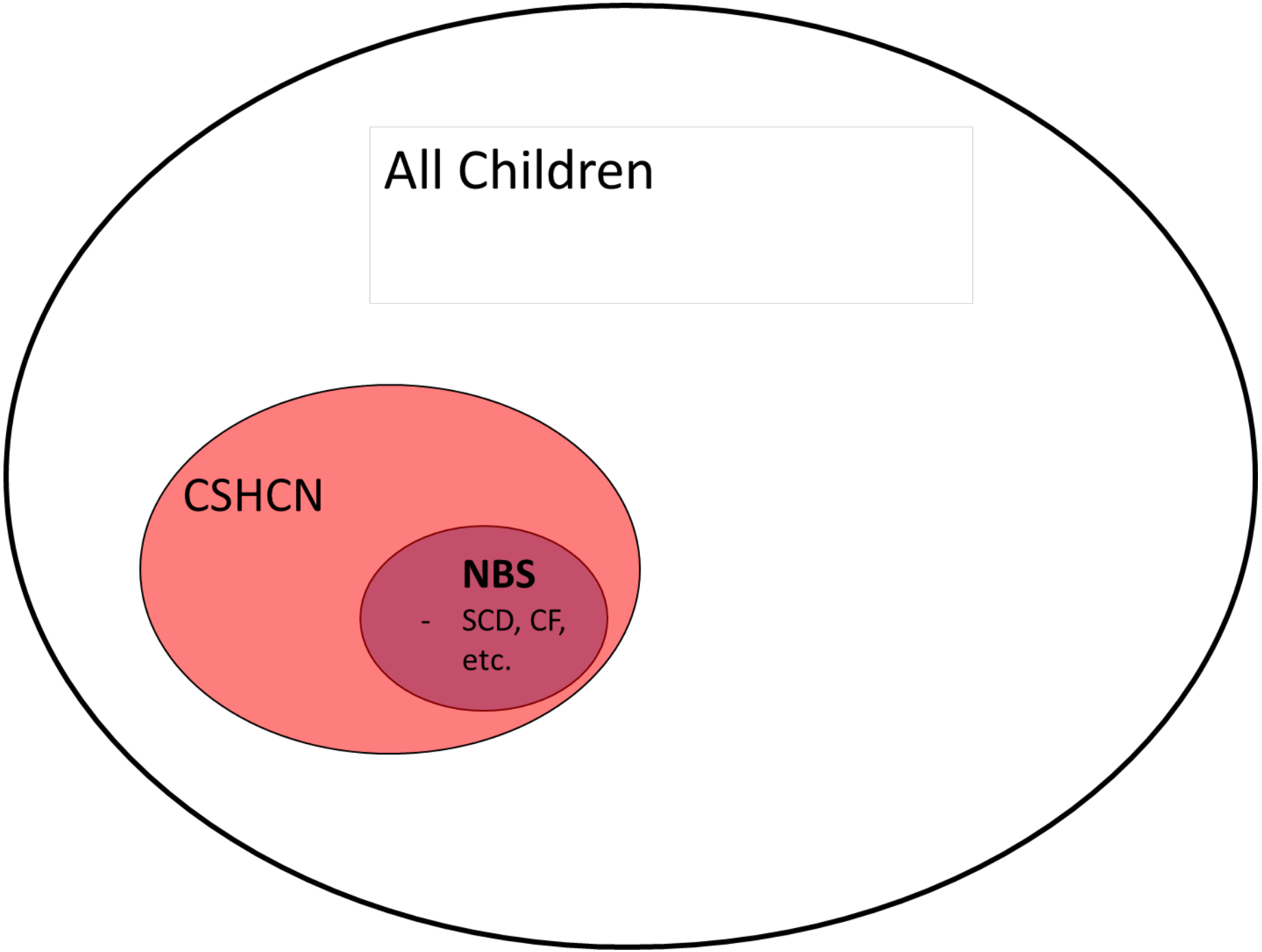
- Many different types of Quality Measures
  - Process - did something happen? (e.g. vaccine given)
  - Access to care - are patients able to get the care? (e.g. physician communicates well)
  - Health outcomes - hospitalizations, HGB A1C level, stroke
  - Quality of life - school attendance, perception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


All Children
CSHCN
NBS
- SCD, CF, etc.

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