

Follow-up and Treatment Workgroup Progress Report

ACHDNC Meeting, May 12, 2017

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Two Sub-workgroups

- Medical Foods for Inborn Errors of Metabolism
 - Report affirmed by ACHDNC
 - Publication planned
- Quality Measures for Long-term Follow-up
 - Quick history of ACHDNC activity
 - Progress report – Alan Zuckerman

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

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, MPH¹, Lisa Feuchtbaum, DrPH, MPH², Christopher A. Kus, MD, MPH³, Alex R. Kemper, MD, MPH⁴, Susan A. Berry, MD⁵, Jill Levy-Fisch, BA⁶, Julie Luedtke, BS⁷, Celia Kaye, MD, PhD⁸, and Coleen A. Boyle, PhD, MS¹

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

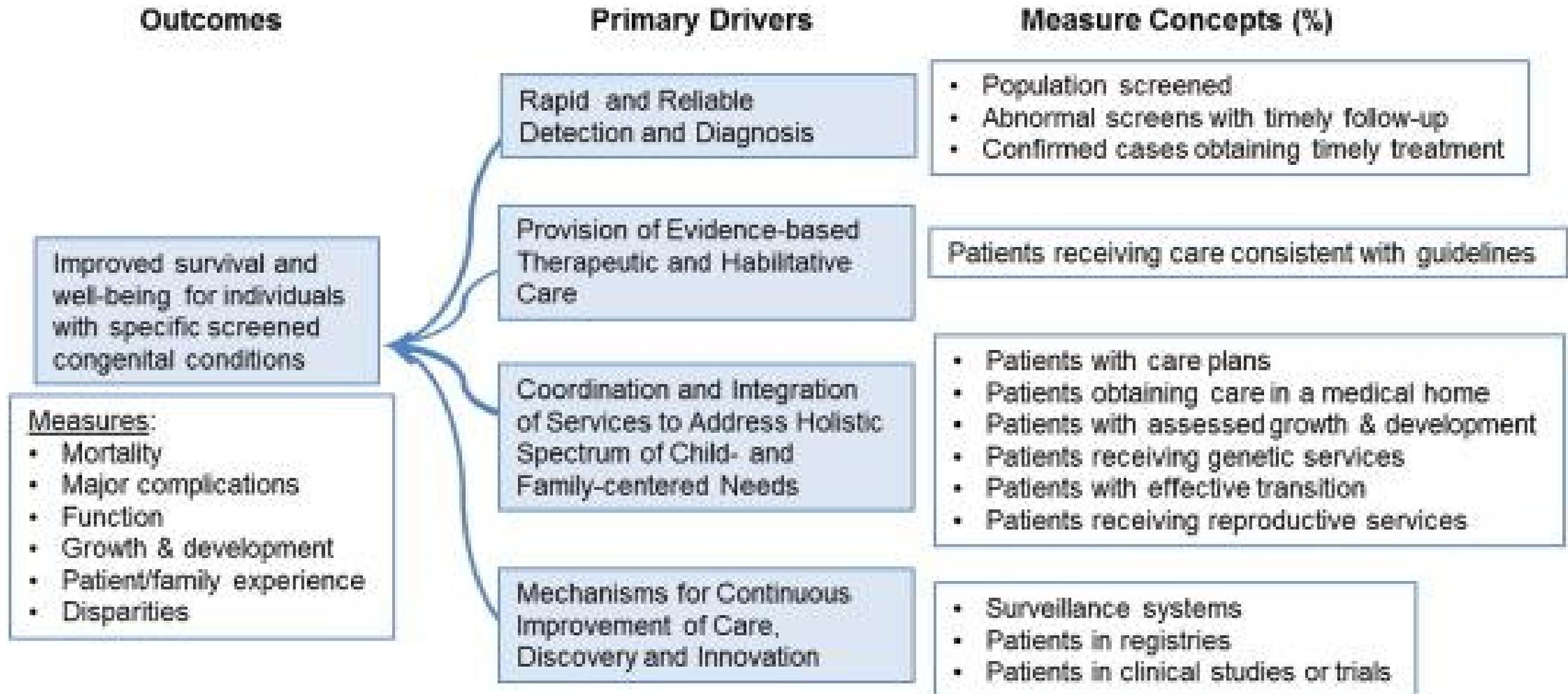
ACHDNC – Molecular Gen & Metab (2016)

A framework for assessing outcomes from newborn screening: on the road to measuring its promise☆



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Lisa Feuchtbaum ^f, Susan A. Berry ^g, Anne Marie Comeau ^h, Bradford L. Therrell ⁱ, Amy Brower ^j,
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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



February and May 2016 ACHDNC Meetings

Role of clinical quality measures to promote LTFU

The Workgroup identified potential ways to address this priority

- Draft a background document to describe what is known about quality measures and newborn screening, and identify areas of need and possible opportunities to use clinical quality measures in long-term follow-up.
- Develop case studies that provide examples of how quality measures are used in newborn screening – describe success or barriers.
- Describe other key findings (e.g. use of quality measures vs performance measures, and different approaches for disease-specific measures vs public health services vs patient/child specific measures).
- Summarize key findings

The Role of Clinical Quality Measures to Promote Long-Term Follow-up Progress Report

May 12, 2017

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We are Making Progress in Addressing the Charge to the Workgroup

- Review the state of the art of clinical quality measures
 - Quality measures are gaining increased attention for value based reimbursement and Maintenance of Certification.
 - New standards are facilitating their use.
 - They are a first step in quality improvement and a pathway to new knowledge acquisition.
- Assess the use of clinical quality measures for newborn screening
 - Use has been limited with rare conditions, but the importance of quality measures has been demonstrated for cystic fibrosis, sickle cell disease, and other conditions.
 - Substantial gaps and barriers must be overcome
- Assemble case studies illustrating the use of clinical quality measures
 - We have collected several examples that illustrate the value of quality measures for newborn screening and challenges faced.
 - This meeting we heard from the AHRQ Pediatric Quality Measures Program and a University of Maryland study of Primary Care and Long Term Follow-up of Newborn Screening.

Can Quality Measures be Used for Long-Term Follow-up of NBS?

- This current project is a continuation of 10 years of prior work by the Long Term Follow-up and Treatment subcommittee of the ACHDNC.
 - The Hinton papers on Key Questions for LTFU of NBS and A Framework for Long Term Follow-up are a good fit with the current approaches to quality measures.
 - The 2008 Kemper definition of Long Term Follow-up remains valid and can drive quality measures.
- We can learn lessons from conditions not covered by newborn screening such as Asthma and ADHD that illustrate the role quality measures can play in long term follow-up.
- Quality measures limit the ask for data and seek to answer specific questions.
- Long term follow-up is about more than collecting data, it is about providing and changing care.
- It is important to find measures that matter and have reliable data.

What are the Gaps and Barriers in Applying Quality Measures to Newborn Screening?

- Are there Gaps in Evidence?
 - This has been a concern because many conditions have sub-types and the best treatment is not always clear for some conditions.
 - There are many generic consensus measures that can apply to all conditions.
 - Cystic Fibrosis demonstrated that measures are a pathway to evidence.
- Are there Gaps in Developing Measures?
 - Developing measures is a challenge for rare disorders with late onset.
 - The NQF certification process is difficult for newborn screening.
 - Validating measures is costly.
 - The lack of pediatric quality measures led to the CMS/AHRQ PQMP mandated by CHIP-RA
- Are there Gaps in Adopting and Using Measures?
 - The cost of data collection, and small numbers of patients in a single practice are challenges – we must integrate quality measures into routine care.
 - Measures for Sickle Cell disease are now available and expected to increase in use.
 - Some models used by health departments are hard to replicate elsewhere.

There is a Need to Move Beyond Disease Specific Measures

- Traditional approaches to quality measurement may fall short for newborn screening.
- We need to include Public Health or Newborn Screening System measures to track that services are available, that individuals are not lost to follow-up, and transition into adult care.
- We need child-specific measures that focus on access to medical homes, available treatment, child well-being, and family satisfaction with the care process.
- Data sources may need to move beyond healthcare providers.

Available Resources Could Accelerate the Use of Quality Measures for Newborn Screening

- Quality measures are hard to do, new tools can make it easier in the future.
- ONC, CMS, and AHRQ have an Electronic Clinical Quality Improvement Resource Center.
 - <http://ECQI.HealthIt.gov>
 - Access to Health IT standards for quality measure definition and reporting
 - Access to available quality measures and incentive programs
- NewSTEPS case definitions and care reporting databases can help define the denominator for NBS quality measures.
- NBSTRN has an LPDR database with definitions of data fields and including core and public health variables.

Sub-workgroup is at a cross-roads

- Sub-workgroup has completed its task from spring 2016
 - Background document on quality measures and NBS (areas of need and opportunity)
 - Case studies that provide examples of how quality measures
 - Other findings: disease-specific measures vs public health services vs patient/child specific measures
- Recommendation
 - Finalize the report on clinical quality measures for August 2017 meeting
 - Over next few months, work on specific suggestions for next steps for the sub-workgroup to present as possibilities to the ACHDNC