# Follow-up and Treatment Workgroup Progress Report

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# 2017 Follow-up and Treatment Workgroup

#### **ACHDNC MEMBERS**

- Jeffrey P. Brosco, MD, PhD (FUTR Chairperson)
- Susan A. Berry, MD
- Kamila B. Mistry, PhD, MPH
- Annamarie Saarinen

#### **ORGANIZATION REPRESENTATIVES**

- Carol Greene, MD
   Society for Inherited Metabolic Disorders
- Christopher A. Kus, MD, MPH (FUTR Co-Chair)
   Association of State & Territorial Health Officials
- Robert J. Ostrander, MD
   Family Practice Physician

#### **WORKGROUP MEMBERS**

- Amy Brower, PhD
- Christine S. Brown, MS
- Kathryn Camp, MS, RD
- Debra Freedenberg, MD, PhD
- Kathryn Hassell, MD
- Nancy Doan Leslie, MD
- Sylvia Mann, MS, CGC
- Jana Monaco
- Margie A. Ream, MD, PhD
- Joseph H. Schneider, MD, MBA, FAAP
- Janet Thomas, MD
- Alan E. Zuckerman, MD, FAAP

# Proposed Future Workgroup Activities

- Environmental scan follow the work of Kemper and Lam et al and contribute information
- 2. Create a "Roadmap" to a practical system of NBS LTFU
  - "L" = "long-term," "longitudinal," "lifespan"
- 3. Medical Foods for Inborn Errors of Metabolism publication
- 4. Quality Measures complete report; consider options for next steps

# Two Sub-workgroups (Activities 3 and 4)

- Medical Foods for Inborn Errors of Metabolism
  - Report completed, final stages of editing
  - Publication planned; Sue Berry, lead (Feb)
- Quality Measures for Long-term Follow-up
  - Alan Zuckerman (lead), Carole Greene, Kamila Mistry,
     Margie Ream, and many more
  - Final report ACHDNC review (Feb)
    - Revise "Executive Summary" (Nancy, Carole, Joe)
    - Clarify "next steps" section (entire workgroup)
    - Add final pieces (tables, appendices, etc.)

# Workgroup Activity 1: Environmental Scan

## 1. Intended Purpose

 Document current activities and identify key gaps in long-term follow-up of NBS conditions (provide information for stakeholders) – contribute knowledge to ongoing efforts in this area (Kemper et al) and utilize the environmental scan to help frame future work

## 2. Need, gap, or barrier/challenge the activity is addressing

- Many LTFU activities; many gaps no one person has "whole picture"
- (medical foods and quality measures sub-workgroups, our 10/26/17 conference call, combined experiences of the group)

# 3. Type of Activity and/or Intended Final Product

Collaborate with Kemper/Lam team to help ensure best work product

## 4. Estimated Timeline

• July 2018

# Roadmap to a "Federated System" of LTFU (treatment)

#### Outcomes

### **Primary Drivers**

#### Measure Concepts (%)

Rapid and Reliable Detection and Diagnosis

- Population screened
- · Abnormal screens with timely follow-up
- · Confirmed cases obtaining timely treatment

Improved survival and well-being for individuals with specific screened congenital conditions Provision of Evidence-based Therapeutic and Habilitative Care

Patients receiving care consistent with guidelines

#### Measures:

- Mortality
- Major complications
- Eunction
- Growth & development
- · Patient/family experience
- Disparities

Coordination and Integration of Services to Address Holistic Spectrum of Child- and Family-centered Needs

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- Patients with care plans
- Patients obtaining care in a medical home
- Patients with assessed growth & development
- Patients receiving genetic services
- Patients with effective transition
- Patients receiving reproductive services
- · Surveillance systems
- · Patients in registries
- Patients in clinical studies or trials

Mechanisms for Continuous Improvement of Care, Discovery and Innovation

Hinton et al, 2016

# Workgroup Activity 2: Roadmap

## 1. Intended Purpose

 Provide NBS stakeholders with a roadmap to achieving a "federated system" for long-term/longitudinal/lifespan follow-up

## 2. Need, gap, or barrier/challenge the activity is addressing

- There are many LTFU activities; there are also many gaps
- There is no "system" connecting various activities into a coherent LTFU

## 3. Type of Activity and/or Intended Final Product

- Work with stakeholders to develop a report ("roadmap") with specific roles
- Consider interim steps (e.g. explore how to support patient registries)

## 4. Estimated Timeline

December 2018