# Follow-up and Treatment Workgroup Progress Report

ACHDNC Meeting, November 10, 2018

Jeffrey P. Brosco MD PhD

Mailman Center for Child Development, University of Miami

Florida Department of Health

# FUTR Workgroup Members

Jeffrey P. Brosco, MD, PhD - Workgroup Chair · Christopher A. Kus, MD, MPH - Workgroup Co-Chair

ACHDNC Members Organization Representatives

Susan A. Berry, MD Carol Greene, MD

Kamila B. Mistry, PhD, MPH Robert J. Ostrander, MD

Annamarie Saarinen Debra Freedenberg, Md, Phd

### **Workgroup Members**

Sabra Anckner, RN, BSN Sylvia Mann, MS, CGC

Amy Brower, PhD Dawn S. Peck, MS, CGC

Christine Brown, MS Margie A. Ream, MD, PhD

Nancy Doan Leslie, MD Joseph H. Schneider, MD, MBA, FAAP

Kathryn Hassell, MD Janet Thomas, MD

## Vision for (Long-Term) Follow-Up and 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 Patients with care plans
 Patients obtaining care in

- Patients obtaining care in a medical home
   Patients with assessed growth & development
- Patients with assessed growth & development
- Patients receiving genetic services
- Patients with effective transition
- · Patients receiving reproductive services

Mechanisms for Continuous Improvement of Care, Discovery and Innovation

- Surveillance systems
- · Patients in registries
- Patients in clinical studies or trials

Hinton et al, 2016

# Workgroup Activities

- 1. Quality Measures
  - Report complete and will be posted
  - Dissemination plans on-going (Alan Zuckerman)
- 2. Medical Foods for Inborn Errors of Metabolism
  - Report complete (final edits)
  - Plans for publication in abbreviated format (Sue Berry)
- 3. Environmental scan Kemper and Lam et al
  - Who is doing what, using which tools
- 4. Create a "Roadmap" to a practical system of NBS LTFU
  - "L" = "long-term," "longitudinal," "lifespan"

## Workgroup Activity: 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

# Follow-up and Treatment Workgroup

## PHSI Feedback

ACHDNC Meeting, November 10, 2018

Jeffrey P. Brosco MD PhD

Mailman Center for Child Development, University of Miami

Florida Department of Health

## Public Health **System** Impact Assessment

- Who is answering the survey?
  - NBS program/lab good people who don't want to say "no" to babies
    - Is there a way to distinguish later vs. early adopters?
  - Include state advisory board, public health leadership
- "Time" seems less useful tool because of legislation
  - E.g. question 5 re funding (<1, 1-3, >3 years to resolve funding challenge)
  - Mandates/timetables re RUSP
  - If something is politically important, then time contracts
  - Answers represent a snapshot in time
- Follow-up and treatment Items are on the survey but somehow not fully appreciated
  - Consider separating the survey sections into lab and non-lab issues
  - (Don't forget "point of care")

# Public Health System Impact

- Different audiences and purposes?
  - Primary purpose is to help inform ACHDNC decision
  - Also can be helpful for stakeholders to understand how easy/hard it is to implement a new condition in a state's NBS system
- Public health system impact "big" question?
  - Maybe answers to specific items now on the survey less important than the exercise of trying to imagine what might make it difficult to implement a new condition
  - Given your state's experience with adding new conditions, how hard will it be to add condition x NBS?
  - 1. Technically hard/easy to screen ("flip a switch" vs. entirely new process)
  - 2. How many infant will need follow-up? (prevalence, FPs, indeterminate)
  - 3. Clinical resources for treatment (specialists, funding)
  - 4. Is it a public health priority? What are the opportunity costs? (public health crises, fits into broader strategic planning)

# Public Health System Impact

- Why the "big" question is hard to answer . . .
- The "practical" issues of adding a condition to the state NBS system (testing procedure, follow-up, clinical resources, public health priority) will always need to be interpreted in the context of
  - Resources available
  - Political environment
  - Etc.