

Follow-up and Treatment Workgroup Progress Report

ACHDNC Meeting, November 9, 2017

Jeffrey P. Brosco MD PhD

Mailman Center for Child Development, University of Miami

Florida Department of Health

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

1. 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)

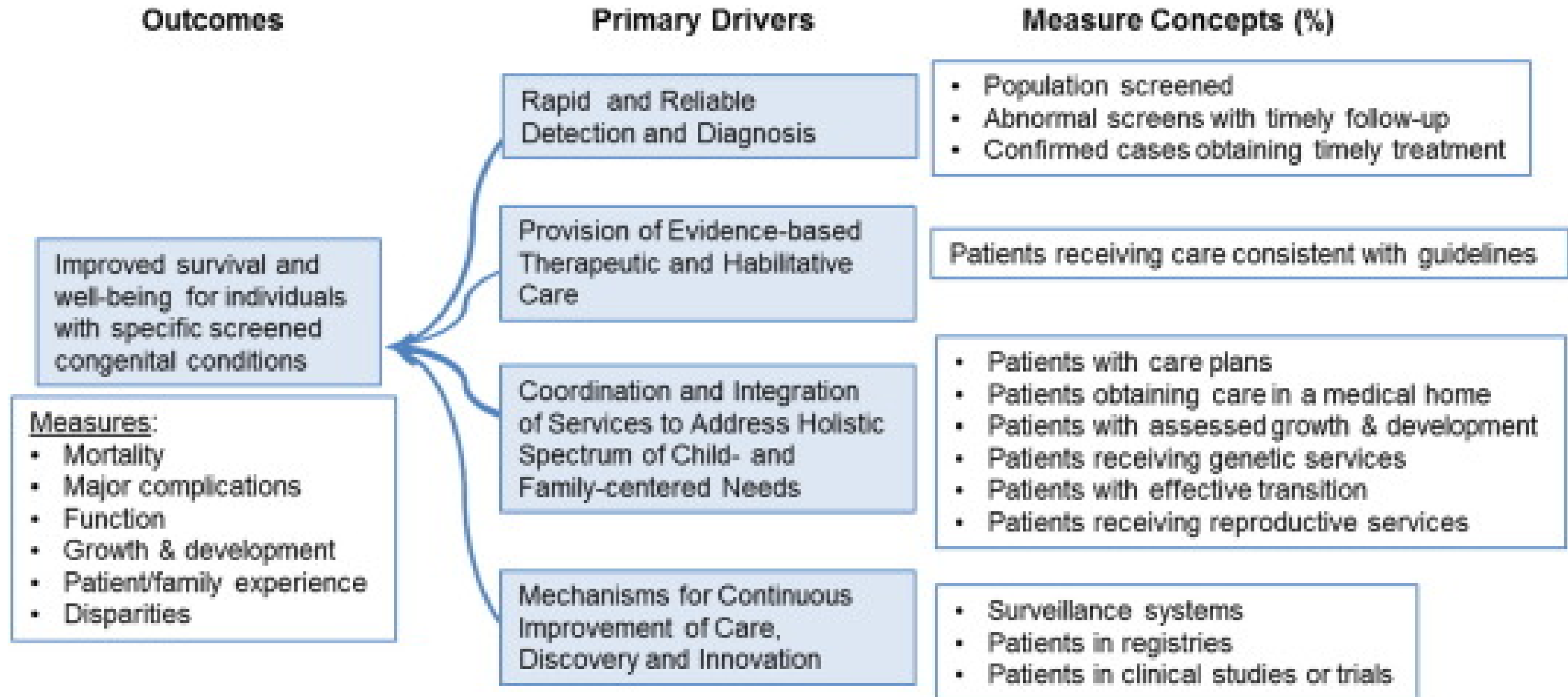
3. **Type of Activity and/or Intended Final Product**

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

4. **Estimated Timeline**

- **July 2018**

Roadmap to a “Federated System” of LTFU (treatment)



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**