Follow-up and Treatment Workgroup Progress Report

ACHDNC Meeting, November 10, 2018

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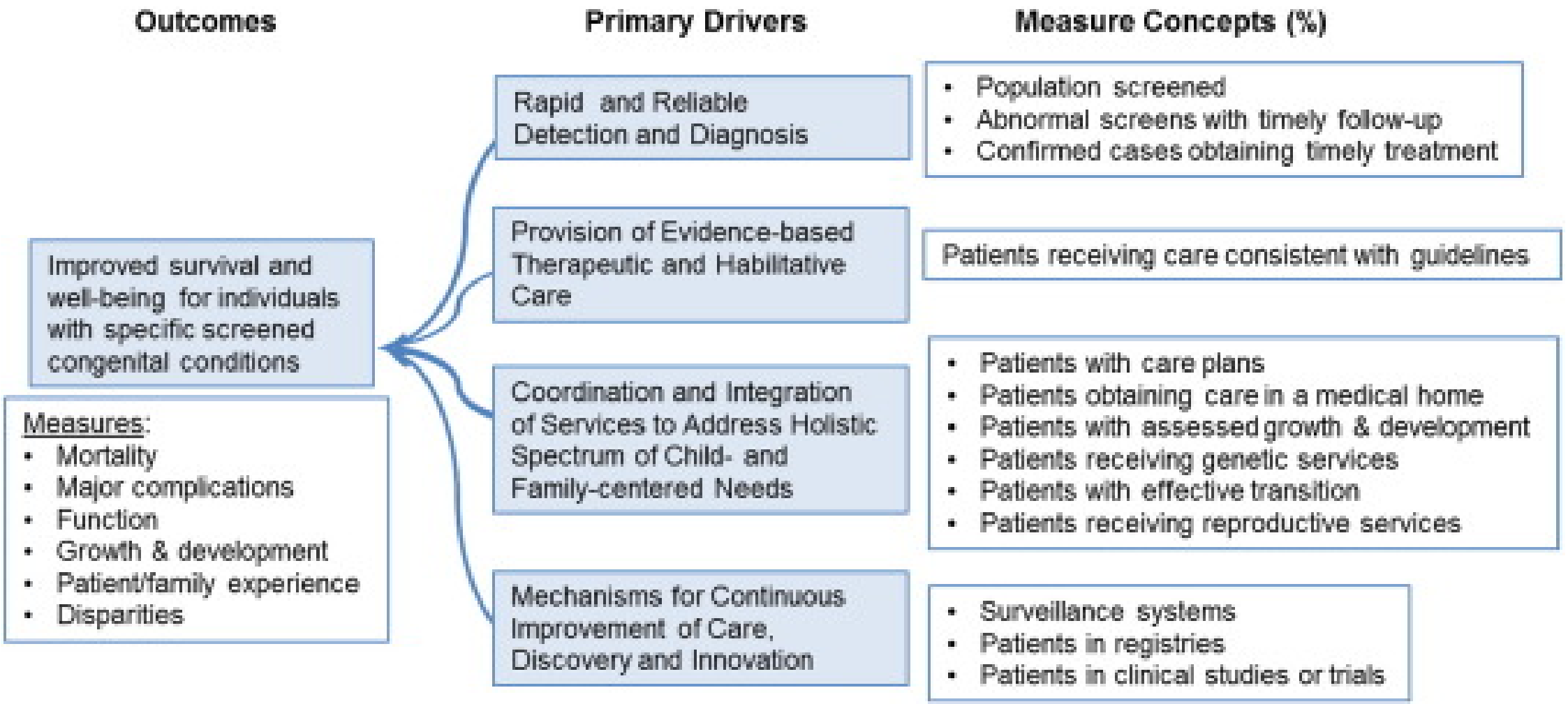
Vision for (Long-Term) Follow-Up and Treatment



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**

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PHSI Feedback

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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.