Follow-up and Treatment Workgroup Progress Report

ACHDNC Meeting, November 10, 2018

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

Outcomes

Improved survival and well-being for individuals with specific screened congenital conditions

Primary Drivers

- Rapid and Reliable Detection and Diagnosis
  - Population screened
  - Abnormal screens with timely follow-up
  - Confirmed cases obtaining timely treatment
- Provision of Evidence-based Therapeutic and Habilitative Care
- Coordination and Integration of Services to Address Holistic Spectrum of Child- and Family-centered Needs
- Mechanisms for Continuous Improvement of Care, Discovery and Innovation

Measure Concepts (%)

- Patients receiving care consistent with guidelines
- 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

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.