



Long Term Follow-Up for X-ALD, MPS I, and Pompe Disease

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Long Term Follow-Up Supervisor

*“Part of the struggle in the past has been that we’re working in silos. We’re all working toward the same thing here. Let’s figure out how to do this together.” —
Sister Lillian Murphy,
Mercy Housing*

What questions should LTFU be able to answer

Hinton et al. *Genet Med.* 2011:13(11)

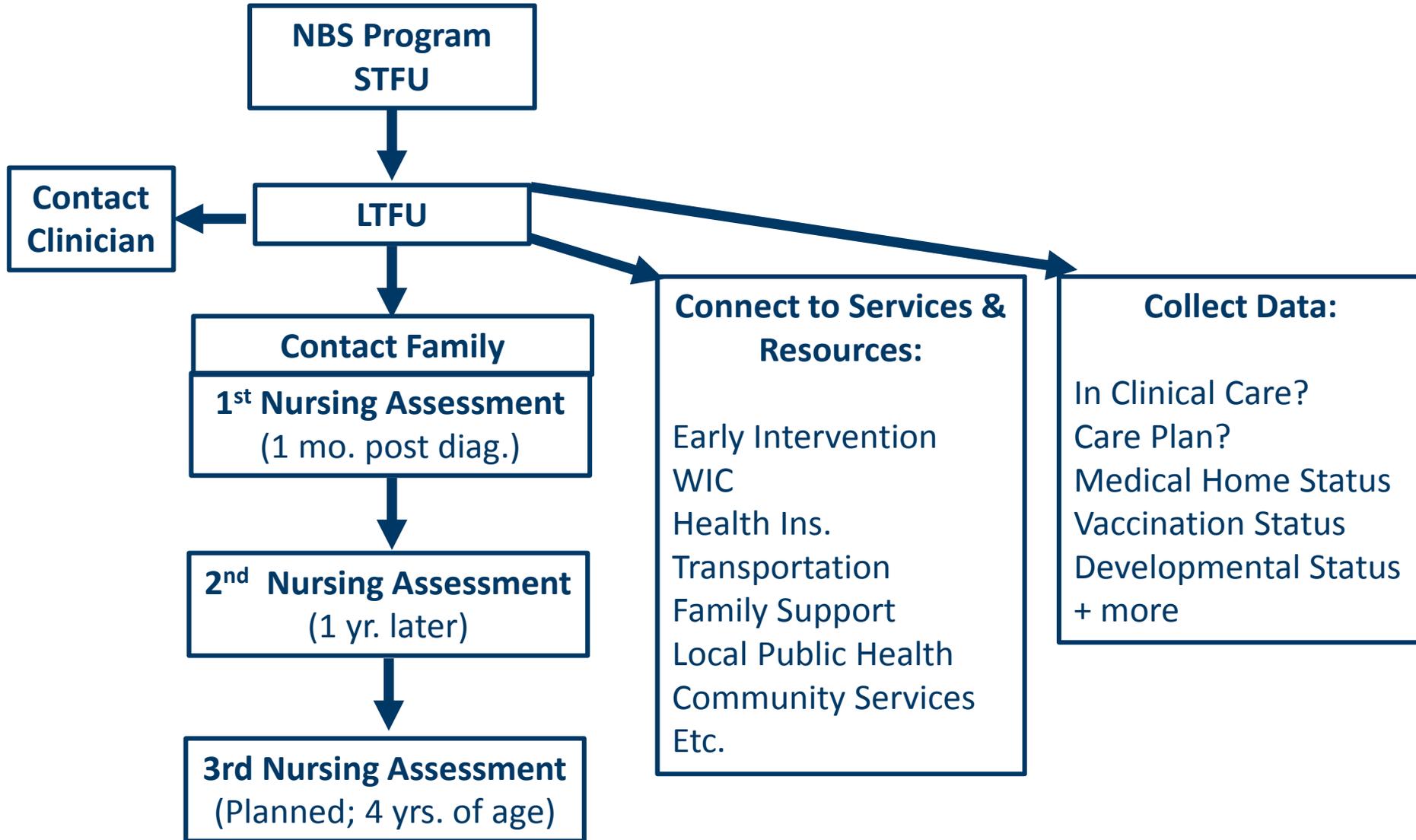
- **Do children/adolescents receive coordinated care through a medical home?**
 - What percentage of children/adolescents has a family-centered medical home?
 - Is there capacity to provide services and do children/adolescents have financial access to services?
- **How are the children/adolescents doing clinically?**
 - What are developmental, physical, and mental outcomes among affected children?
 - Are service providers using best practices?

What questions should LTFU be able to answer

Hinton et al. *Genet Med.* 2011:13(11)

- **How do we assure ongoing QI?**
 - Are there policies in place at the state/national level that facilitate collection and exchange of information among all components of the NBS system?
- **What clinical and observational long-term follow-up research efforts are being performed at the state and national levels?**

LTFU in Minnesota

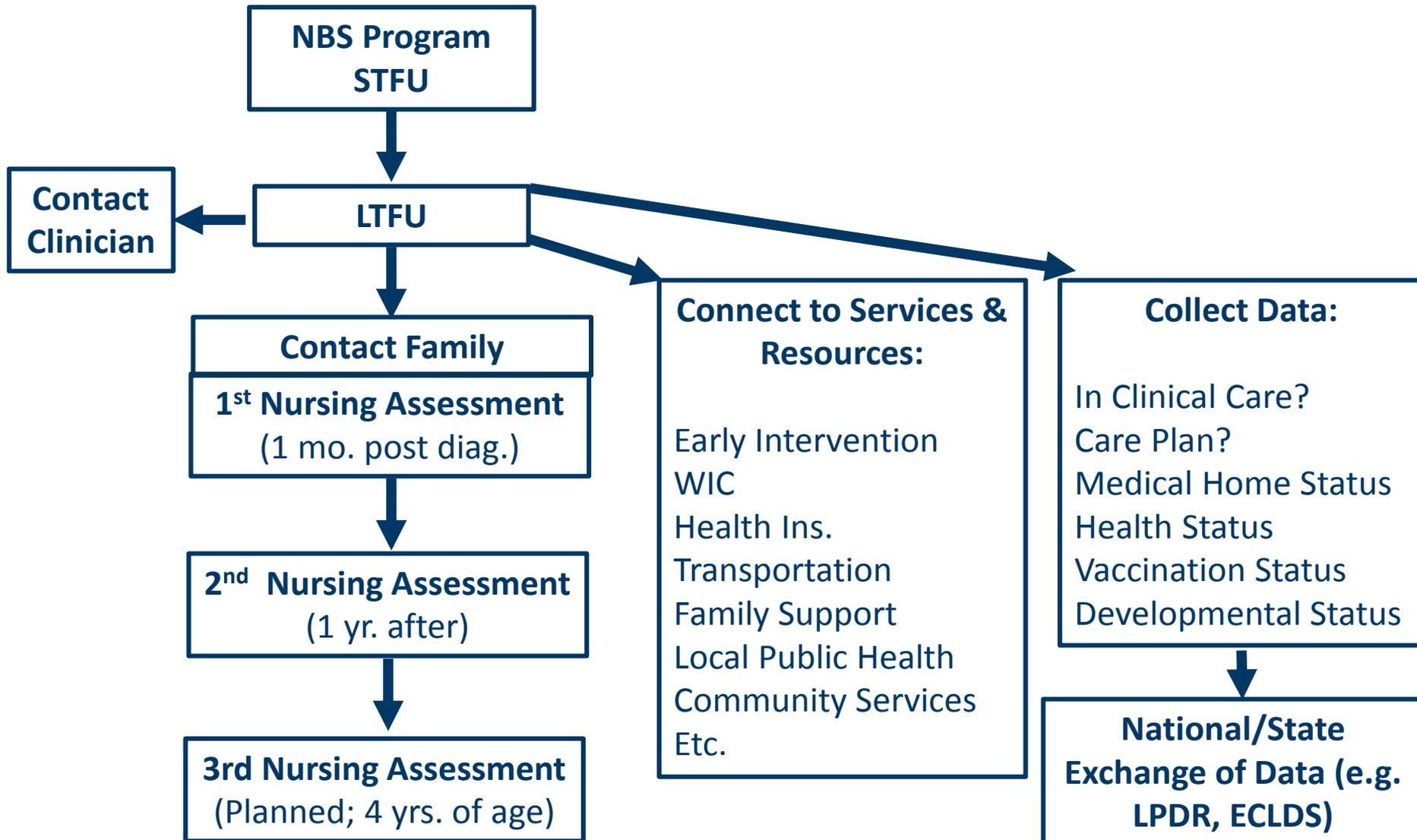


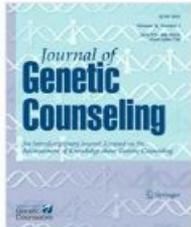
What questions should LTFU be able to answer

Hinton et al. *Genet Med.* 2011:13(11)

- **Do children/adolescents receive coordinated care through a medical home?**
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- **How do we assure ongoing QI?**
- **What clinical and observational long-term follow-up research efforts are being performed at the state and national levels?**

LTFU in Minnesota





[Journal of Genetic Counseling](#)

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Congenital Hypothyroidism Long-Term Follow-up Project: Navigating the Rough Waters of a Multi-Center, Multi- State Public Health Project

Authors

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Kupper Wintergerst , Gina Gembel, Tracey Kreipe, Patrick Zeller, Erica Eugster, Bill Young, Karen Andruszewski, Mary Kleyn, Troi Cunningham, Sandy Fawbush, Nancy Vanderburg, Joe Sockalosky, Ram Menon, Sharon Linard, Gary Hoffman, Lisa Gorman



Molecular Genetics and Metabolism

Volume 119, Issues 1–2, September–October 2016, Pages 75–82



221 newborn-screened neonates with medium-chain acyl-coenzyme A dehydrogenase deficiency: Findings from the Inborn Errors of Metabolism Collaborative

Kristi Bentler^a, Shaohui Zhai^b, Sara A. Elsbecker^c, Georgianne L. Arnold^d, Barbara K. Burton^e, Jerry Vockley^d, Cynthia A. Cameron^b, Sally J. Hiner^b, Mathew J. Edick^b, Susan A. Berry^{a,1,2}  

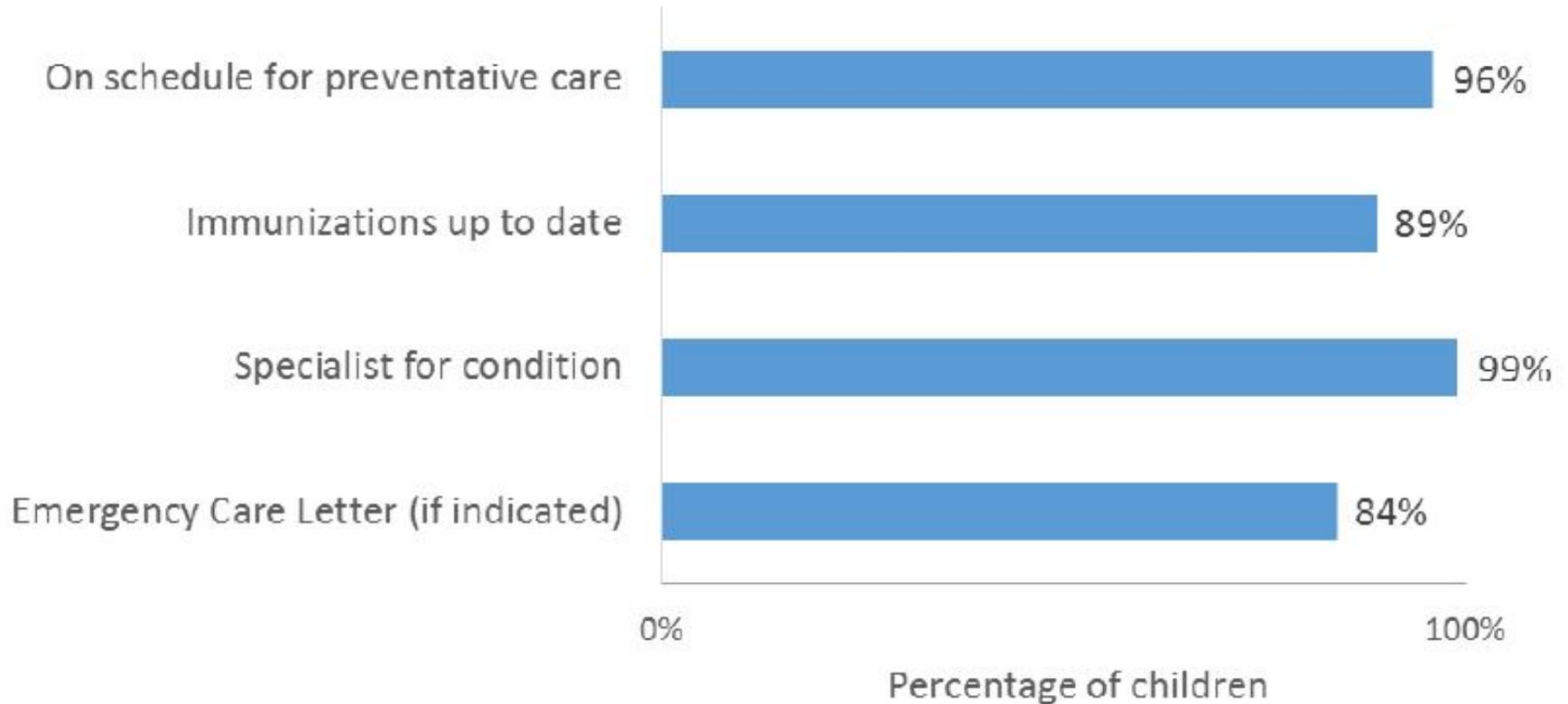
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<https://doi.org/10.1016/j.ymgme.2016.07.002>

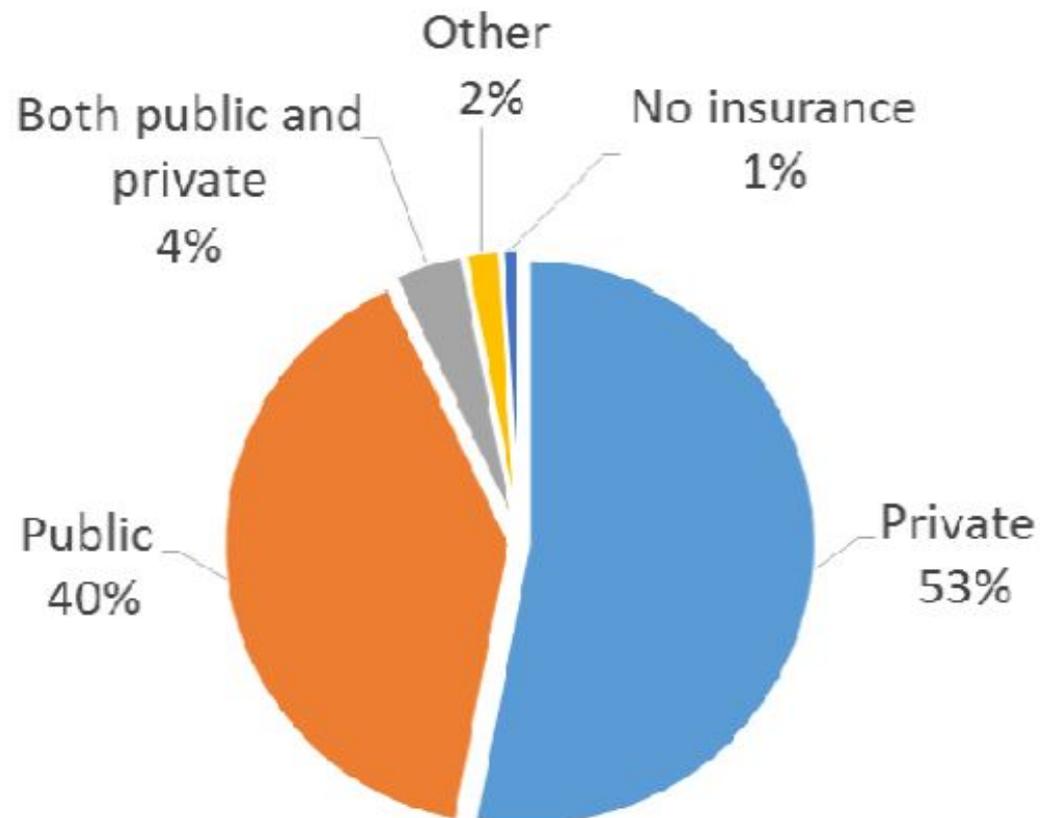
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Minnesota Children with a Bloodspot Condition, 2012 – 2015

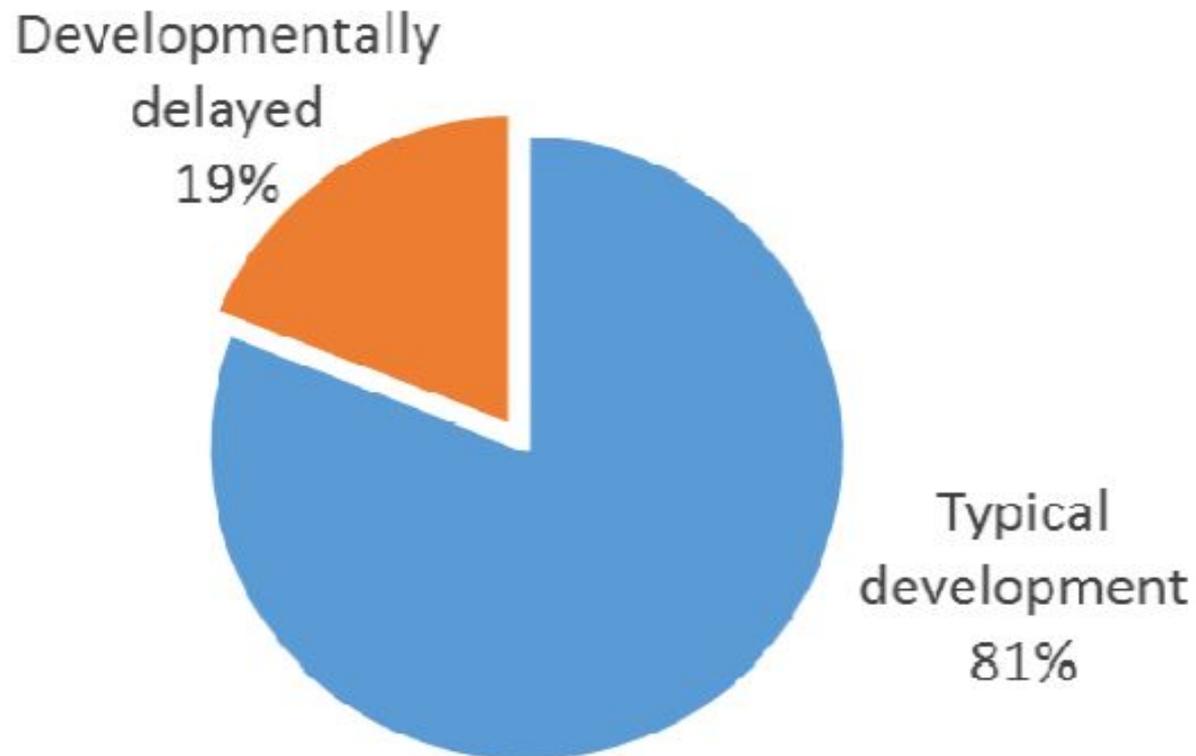
Health Care Status



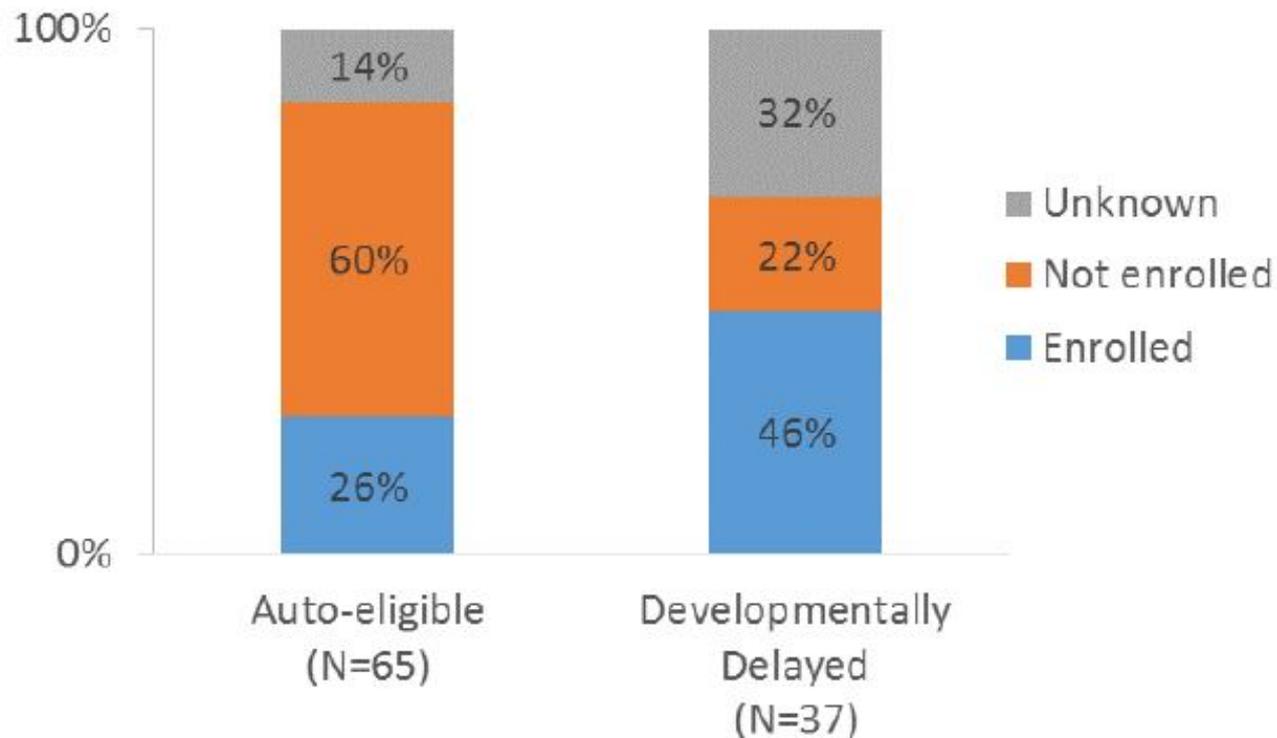
Health Insurance Status



Developmental Status as Reported by Parent/Guardian



Part C Early Intervention Services Enrollment Status for Children who are Auto-eligible or Developmentally Delayed



Underscoring the need for robust LTFU

Condition	Gaps/Needs	What we're doing
Metabolic Conditions	Medical Formula / Foods	Legislation; Council of Health Plans
Galactosemia in Karen Population	Language; Soy Formula; Transportation; Insurance Status	Work with PCPs & Clinicians on translators; WIC; DHS
Sickle Cell Disease	Underinsured; Transportation; Access to opioids; Lack of Family Support	DHS; Emergency Departments; \$\$\$ and TA to Community Based Organizations

LTFU for the New Disorders

What is the plan for LTFU for the new disorders in MN?



Tentative Plan – MAY CHANGE NEXT WEEK?

Phase 1

For Early Infantile Forms - start with long term follow-up as usual

1. Reach out to the treating clinician
2. Call the family for a 1st nursing assessment
3. Connect family to resources / services, collect data on health status, care coordination, family needs

Phase 2

Work on protocols for late onset forms – focus of MN NewSTEPs grant

1. Identify and convene stakeholders and partners
2. Develop pilot protocols for follow-up
3. Test the protocols

Phase 3

Finish protocols under the MN NewSTEPs grant for early infantile and late onset forms

1. Apply continuous quality improvement to Phases 1 and 2
2. Finalize protocols
3. Deliver to NewSTEPs

What we (think) we need to know

- For Infantile Onset forms
 - What do families need that's different from what we currently do?
- For Late Onset forms
 - What is a late onset form? (1 year old Pompe???)
 - What follow-up / monitoring do they need?
 - What is the role of public health?
- For X-ALD – Female Carriers (or Affected Females?)
 - What follow-up / monitoring do they need?
 - What is the role of public health?

54 Models for 54 Programs

- Define your Question – What do you want to do for LTFU?
- Ask the families and the clinicians – what are their needs in order to get the best possible outcomes?
- What are existing resources? MCH? Clinical Centers? Medical Homes?
- Existing Data Sources to tap into?
 - Data, Data, Data – we need to do a better job of publishing!!!
- As a state employee, I can't lobby.....but I can provide “Technical Assistance” to folks that promote LTFU to the state legislature