University of Michigan
C.S. Mott Children's Hospital

Child Health Evaluation and Research Unit
University of Michigan

Assessing the Long-Term Follow-Up (LTFU) Data Collection Activities of State Newborn Screening Programs

Beth Tarini, MD, MS
Shelby Lemke, BA

DACHDNC meeting
September 19-20, 2013

Confidential: Do not distribute

BACKGROUND

WHEN DOES NBS LTFU BEGIN?

After a newborn with an out-of-range NBS screening result has received diagnostic confirmation of a disorder

BACKGROUND

WHAT ACTIVITIES ARE INVOLVED IN NBS LTFU?

"includes assurance and provision of quality chronic disease management, condition-specific treatment, and age-appropriate preventive care throughout the lifespan of individuals identified with a condition included in newborn screening."*

*Kemper AR, et al. Long-term follow-up after diagnosis resulting from newborn screening: statement of the US Secretary of Health and Human Services' Advisory Committee on Heritable Disorders and Genetic Diseases in Newborns and Children. Genet Med. 2008 Apr;10(4):259-61.

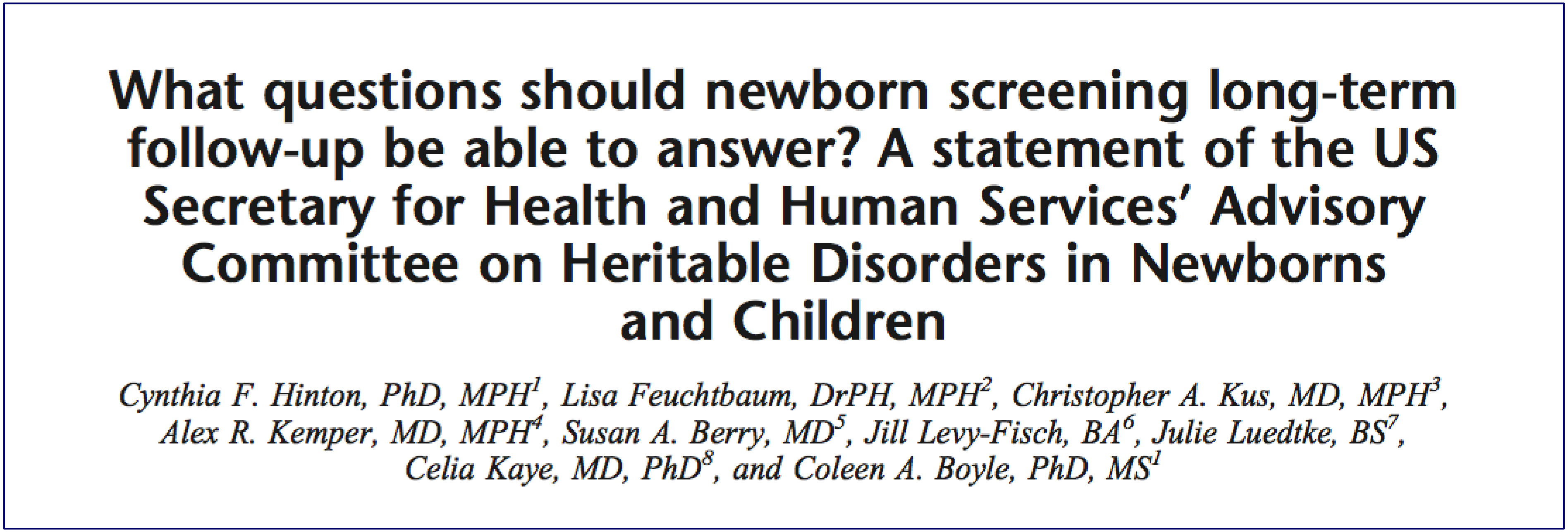
BACKGROUND

SACHDNC GUIDANCE ON LTFU

September 23, 2009
Follow-up and Treatment Subcommittee of the SACHDNC convened a workshop entitled "Overarching Questions in Long-Term Follow-up and Treatment in Newborn Screening"

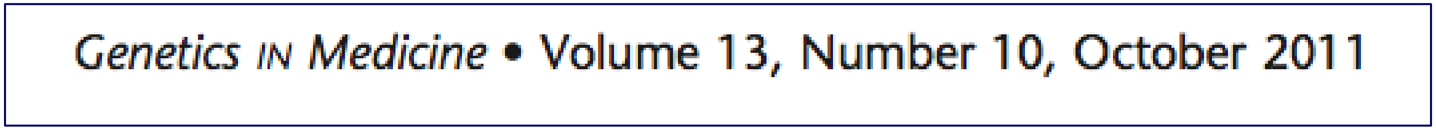
BACKGROUND

SACHDNC GUIDANCE ON LTFU



What questions should newborn screening long-term follow-up be able to answer? A statement of the US Secretary for Health and Human Services' Advisory Committee on Heritable Disorders in Newborns and Children

Cynthia F. Hinton, PhD, MPH[1], Lisa Feuchtbaum, DrPH, MPH[2], Christopher A. Kus, MD, MPH[3], Alex R. Kemper, MD, MPH[4], Susan A. Berry, MD[5], Jill Levy-Fisch, BA[6], Julie Luedtke, BS[7], Celia Kaye, MD, PhD[8], and Coleen A. Boyle, PhD, MS[1]



Genetics IN *Medicine* • Volume 13, Number 10, October 2011

BACKGROUND

SACHDNC GUIDANCE ON LTFU

"SACHDNC sought to identify the most important questions and issues that could be used to inform the development of a NBS-LTFU data system…The questions and issues presented in this report could guide these data standardization activities, leading to quality measures by which LTFU programs can evolve and improve" (Hinton et al.)

OBJECTIVE

Given this SACHDNC guidance, we sought to examine the current status of LTFU data collection activities among state NBS programs

<u>Note</u>:

– Part of a larger project to examine the policy impact of non-RUSP SACHDNC recommendations

– This issue is not up for Committee vote

METHODS

- Study Design
  - Web-based survey
- Recruitment
  - NBS Follow-up Directors
  - Additional participants identified through snowball sampling

METHODS

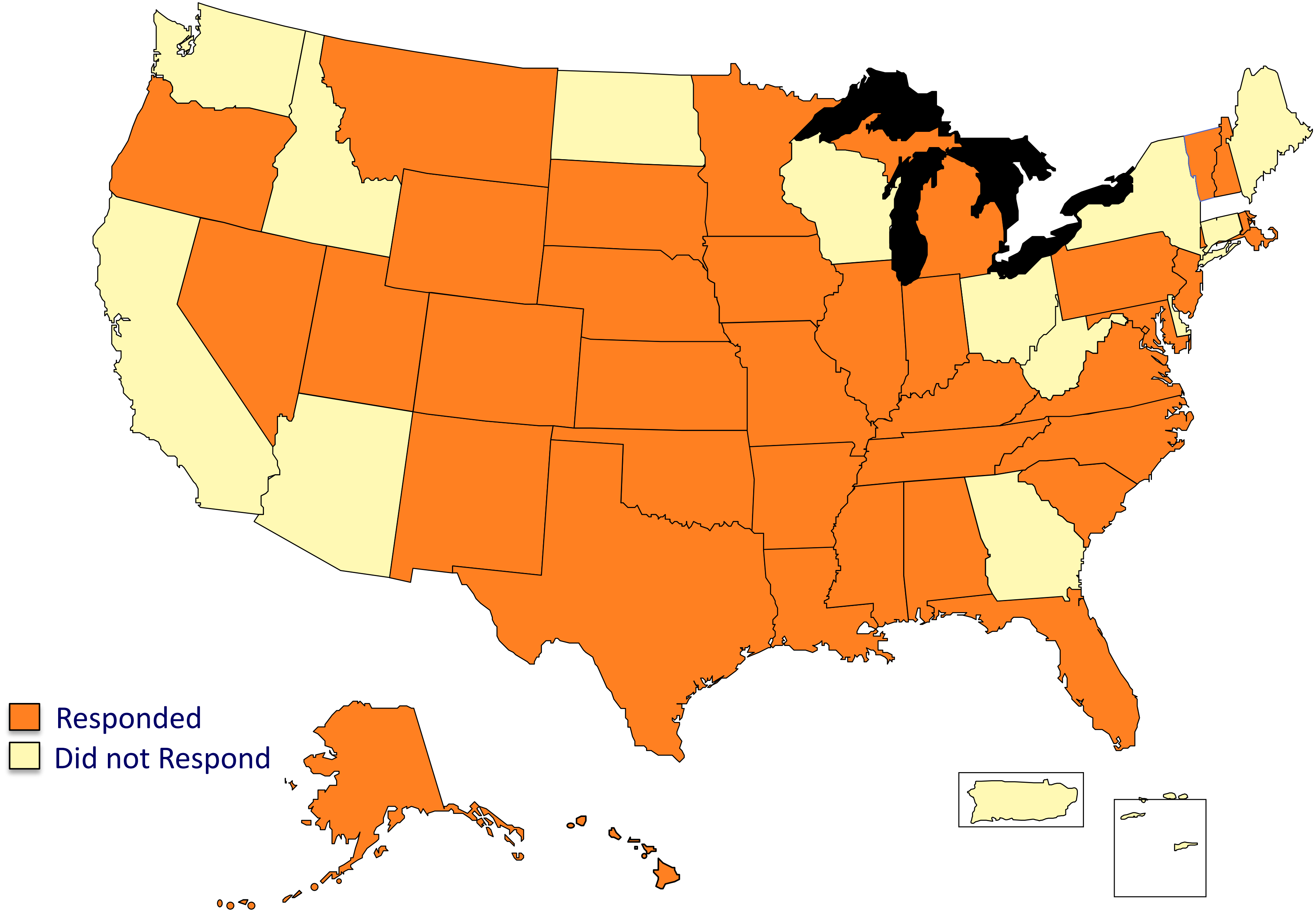
- Data Collection & Verification
  - – July – August 2013
  - – 3 survey waves
  - – Respondents provided opportunity to verify data after collection completed

SURVEY INSTRUMENT

- Development guided by SACHDNC statement and informed by previous studies (Hoff et al.) of NBS programs’ LTFU activities
- Questions addressed 3 major areas
  - LTFU data collection methods, data type & uses
  - Barriers
  - General attitudes

RESULTS

Response Rate = 72%



🟧 Responded

🟨 Did not Respond

NUMBER OF RESPONDENTS

	Complete with 1 respondent	Complete with >1 respondent
Complete= 24	21	3

NUMBER OF RESPONDENTS

	Complete with 1 respondent	Complete with >1 respondent
Complete= 24	21	3

	Incomplete with 1 respondent	Incomplete with >1 respondent
Incomplete= 14	13	1

RESPONDENTS

Title	%
NBS program manager	27.8
Follow-up coordinator	27.8
NBS program director	25.0
NBS program administrator	5.5
Genetics coordinator	2.8
Other	11.1

LTFU DATA COLLECTION

LTFU DATA COLLECTION

Does your program collect, access, or contract out the collection of LTFU data - defined as ANY data for individuals diagnosed with ANY disorder through NBS?

Please select all that apply.

LTFU DATA COLLECTION

Does your program collect, access, or contract out the collection of LTFU data?	**No. of States**
Yes, the NBS program actively collects this type of data	21
Yes, the NBS program accesses existing data that has already been collected by partners, such as public or private entities (e.g., Medicaid, Children with Special Healthcare Needs, WIC, Title V programs, etc.)	7
Yes, the NBS program contracts out the collection of this type of data to partners, such as public or private entities (e.g., Medicaid, Children with Special Healthcare Needs, WIC, Title V programs, etc.)	6
No, the NBS program has no method of collecting data, accessing data, or receiving this type of data from contracted entities	12

Has your program used the following article authored by SACHDNC* to guide their development of these LTFU activities?

Hinton et al. (2011). What questions should newborn screening long-term follow-up be able to answer? A statement of the US Secretary for Health and Human Services' Advisory Committee on Heritable Disorders in Newborns and Children. *Genetics in Medicine, 13(10),* 861-865.

Has your program used the following article authored by SACHDNC* to guide their development of these LTFU activities?

Hinton et al. (2011). What questions should newborn screening long-term follow-up be able to answer? A statement of the US Secretary for Health and Human Services' Advisory Committee on Heritable Disorders in Newborns and Children. *Genetics in Medicine, 13(10),* 861-865.

Yes	39.5 %
No	60.5 %

Has your program used the following article authored by SACHDNC* to guide their development of these LTFU activities?

		Collect LTFU Data?	
		Yes	No
Use SACHDNC statement?	Yes	50%	17%
	No	50%	83%

p = 0.051

LTFU DATA CATEGORIES

1. What kind of LTFU data do states collect?
2. For what purpose?
3. Do they routinely monitor data?

WHAT LTFU DATA DO STATES COLLECT?

Data Category	**Number of States Collecting**
Follow-up Status	25
Patient Demographics	21
Health Care Utilization	21
Treatment Regimen	20
Health Outcomes	17
Patient Access to Services	11
Cost	6
Enrollment in Research Studies	1

DATA CATEGORY: FOLLOW-UP STATUS

Number of states collecting = 25

Purpose	Monitor Routinely
Monitoring/improving clinical care	59%
Connection of patients and families to support services and public health programs	38%
Program Evaluation	35%
Research	50%

Examples: Missed appointments, lost to follow-up (due to change in location, unable to contact, declined follow-up, treatment not necessary, deceased, etc.), etc.

DATA CATEGORY: PATIENT DEMOGRAPHICS

Number of states collecting = 21

Purpose	Monitor Routinely
Monitoring/improving clinical care	19%
Connection of patients and families to support services and public health programs	47%
Program Evaluation	21%
Research	0%

Examples: Basic demographics (name, DOB), race/ethnicity. patient condition/diagnosis, etc.

DATA CATEGORY: HEALTH CARE UTILIZATION

Number of states collecting = 21

Purpose	Monitor Routinely
Monitoring/improving clinical care	33%
Connection of patients and families to support services and public health programs	53%
Program Evaluation	17%
Research	0%

Examples: Information about primary care providers and specialty care providers, referrals for specialist evaluation (e.g., developmental, behavioral, mental health assessment), use of public health programs (WIC, Children with Special Healthcare Needs, Early Intervention, etc.), hospitalizations or ED visits, etc.

DATA CATEGORY: TREATMENT REGIMEN

Number of states collecting = 20

Purpose	Monitor Routinely
Monitoring/improving clinical care	41%
Connection of patients and families to support services and public health programs	21%
Program Evaluation	30%
Research	0%

Examples: Current treatment regimen, changes to treatment regimen, presence of formalized care plan, whether evidence-based treatments are being utilized when available, etc.

DATA CATEGORY: HEALTH OUTCOMES

Number of states collecting = 17

Purpose	Monitor Routinely
Monitoring/improving clinical care	44%
Connection of patients and families to support services and public health programs	36%
Program Evaluation	40%
Research	0%

Examples: Clinical data (physical growth measures, laboratory results, physical exam findings), developmental assessment (e.g. gross motor, fine motor, and cognitive development), immunization status, special education services (e.g., Individualized Education Program or IEP, disability status, death, etc.

DATA CATEGORY: PATIENT ACCESS TO SERVICES

Number of states collecting = 11

Purpose	Monitor Routinely
Monitoring/improving clinical care	38%
Connection of patients and families to support services and public health programs	38%
Program Evaluation	29%
Research	0%

Examples: Insurance status, changes in insurance status, transportation needs, wait time for specialty clinic appointments, etc.

DATA CATEGORY: COST

Number of states collecting = 6

Purpose	Monitor Routinely
Monitoring/improving clinical care	50%
Connection of patients and families to support services and public health programs	33%
Program Evaluation	50%
Research	0%

Examples: Direct medical costs (e.g., hospitalizations, outpatient visits, drug treatments, procedures, diagnostic tests, medical equipment), direct non-medical costs (e.g., transportation costs, special education, home modifications), patient or family time costs associated with treatment, informal caregiver time, etc.

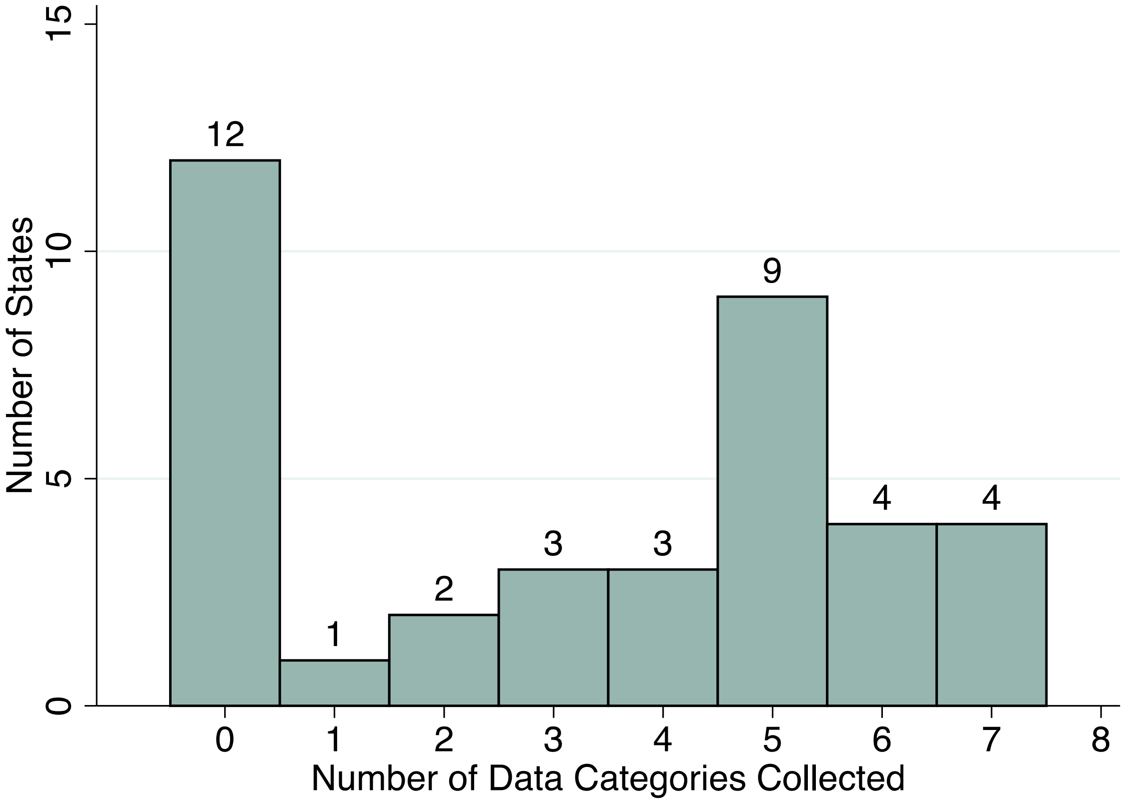
DATA CATEGORY: ENROLLMENT IN RESEARCH STUDIES

Number of states collecting = 1

Purpose	Monitor Routinely
Monitoring/improving clinical care	0%
Connection of patients and families to support services and public health programs	0%
Program Evaluation	0%
Research	0%

Examples: Research studies, clinical trials, etc.

NUMBER OF DATA CATEGORIES COLLECTED



DATA COLLECTION AND STORAGE

COLLECTION METHOD

Which method(s) does your program or its partners use to collect LTFU data?

Please select all that apply.

COLLECTION METHOD

Method*	No. of States
Paper	20
Computerized (e.g., web-based portal, secure email, electronic files, public health databases)	19
Verbal (e.g., telephone)	9
Don't know	0

NOTE: *Could choose more than one. Most common choice was all three modes.

COLLECTION METHOD

Does your program or its partners collect LTFU data directly from hospital or clinic electronic health records?

	% (n)
Yes	21 (4)
No	68 (13)
Don’t know	11 (2)

COLLECTION METHOD

Does your program or its partners collect LTFU data using a web-based portal system?

	% (n)
Yes	47 (9)
No	53 (10)
Don’t know	0 (0)

COLLECTION METHOD

Does your program or its partners collect LTFU data by linking with computerized databases from any of the following entities?

	Yes	No	Don't Know
State or Federal Public Health Programs	37%	5%	5%
State Registries	47%	53%	0%
Other Public Programs	5%	84%	11%

How difficult is it for your program or its partners to collect LTFU data for each of the following disorder categories?

			Less Difficult					More Difficult
		N/A	1	2	3	4	5	6
Critical Congenital Heart Disease (CCHD)	%	65.3	3.9	7.7	3.9	0.0	7.7	11.5
Endocrine	%	7.7	26.9	19.2	23.1	7.7	7.7	7.7
Hearing	%	19.2	19.2	23.1	19.2	7.7	7.7	3.9
Hematologic	%	3.9	19.2	30.7	19.2	7.7	15.4	3.9
Immunologic	%	61.5	3.8	11.5	11.5	3.9	3.9	3.9
Lysosomal Storage Diseases	%	92.3	0.0	3.9	0.0	0.0	0.0	3.9
Metabolic	%	3.9	30.7	38.5	19.2	0.0	7.7	0.0
Pulmonary (e.g. CF)	%	11.5	19.2	30.8	26.9	3.9	7.7	0.0

* Select "N/A" if your program does not screen for one of the disease categories listed

STORAGE METHOD

Which method(s) does your program or its partners use to store LTFU data?

Please select all that apply.

STORAGE METHOD

Method*	No. of States
Paper	12
Computerized (e.g., web-based portal, secure email, electronic files, public health databases)	24
Don't know	1

NOTE: *Could choose more than one.

BARRIERS

WORK PROCESS BARRIERS

States that DO NOT collect LTFU Data

			Less Difficult					More Difficult
		N/A	1	2	3	4	5	6
Communication between physicians and NBS program	%	18.2	9.1	0.0	9.1	18.2	9.1	36.4
Defining specific LTFU data elements to be collected	%	8.3	8.3	0.0	16.7	8.3	33.3	25.0
Identifying a patient's medical home over time	%	8.3	0.0	8.3	25.0	8.3	16.7	33.3
Including newly added disorders in LTFU activities	%	16.7	25.0	0.0	16.7	25.0	0.0	16.7
Management of large computerized databases	%	16.7	8.3	8.3	8.3	0.0	25.0	33.3
Need to obtain parental consent to allow for collection and/or storage of LTFU data	%	8.3	8.3	8.3	25.0	16.7	0.0	33.3
Regulatory requirements for data sharing between agencies or clinics	%	0.0	0.0	16.7	25.0	0.0	16.7	41.7
Variation in LTFU activities needed for different disorders	%	0.0	16.7	8.3	25.0	8.3	8.3	33.3

*You may select "N/A" if the listed item is NOT AT ALL a barrier for your program.

WORK PROCESS BARRIERS

States that DO collect LTFU Data

			Less Difficult					More Difficult
		N/A	1	2	3	4	5	6
Communication between physicians and NBS program	%	23.1	7.7	19.2	23.1	11.5	7.7	7.7
Defining specific LTFU data elements to be collected	%	0.0	11.5	7.7	30.8	3.9	23.1	23.1
Identifying a patient's medical home over time	%	3.9	3.9	19.2	26.9	23.1	15.4	7.7
Including newly added disorders in LTFU activities	%	15.4	26.9	7.7	23.1	7.7	7.7	11.5
Management of large computerized databases	%	7.7	11.5	23.1	23.1	3.9	15.4	15.4
Need to obtain parental consent to allow for collection and/or storage of LTFU data	%	19.2	11.5	15.4	23.1	7.7	15.4	7.7
Regulatory requirements for data sharing between agencies or clinics	%	15.4	11.5	11.5	23.1	19.2	3.9	15.4
Variation in LTFU activities needed for different disorders	%	4.0	12.0	8.0	32.0	16.0	12.0	16.0

*You may select "N/A" if the listed item is NOT AT ALL a barrier for your program.

STRUCTURAL BARRIERS

States that DO NOT collect LTFU Data

			Less Difficult					More Difficult
		N/A	**1**	**2**	**3**	**4**	**5**	**6**
Coordination of LTFU activities with entities outside the NBS program and other public health programs	**%**	33.3	8.3	8.3	8.3	25.0	8.3	8.3
Lack of financial resources for LTFU activities	**%**	8.3	16.7	0.0	0.0	8.3	33.3	33.3
Lack of a designated employee to oversee LTFU activities	**%**	8.3	8.3	0.0	0.0	8.3	25.0	50.0
Lack of statutory authority for program to oversee LTFU activities	**%**	8.3	16.7	0.0	8.3	8.3	8.3	50.0

*You may select "N/A" if the listed item is NOT AT ALL a barrier for your program.

STRUCTURAL BARRIERS

States that DO collect LTFU Data

			Less Difficult					More Difficult
		N/A	1	2	3	4	5	6
Coordination of LTFU activities with entities outside the NBS program and other public health programs	%	34.6	11.5	11.5	7.7	3.9	0.0	30.8
Lack of financial resources for LTFU activities	%	3.9	15.4	3.9	11.5	7.7	23.1	34.6
Lack of a designated employee to oversee LTFU activities	%	19.2	15.4	7.7	7.7	3.9	3.9	42.3
Lack of statutory authority for program to oversee LTFU activities	%	24.0	12.0	12.0	12.0	8.0	8.0	24.0

*You may select "N/A" if the listed item is NOT AT ALL a barrier for your program.

ORGANIZATIONAL CULTURE BARRIERS

States that DO NOT collect LTFU Data

			Less Difficult					More Difficult
		N/A	**1**	**2**	**3**	**4**	**5**	**6**
Lack of clinician interest in LTFU activities	**%**	16.7	8.3	8.3	33.3	25.0	0.0	8.3
Lack of patient and family interest in LTFU activities	**%**	8.3	8.3	16.7	16.7	25.0	0.0	25.0
Lack of NBS program interest in LTFU activities	**%**	25.0	16.7	8.3	8.3	25.0	0.0	16.7

*You may select "N/A" if the listed item is NOT AT ALL a barrier for your program.

ORGANIZATIONAL CULTURE BARRIERS

States that DO collect LTFU Data

			Less Difficult					**More Difficult**
		N/A	**1**	**2**	**3**	**4**	**5**	**6**
Lack of clinician interest in LTFU activities	**%**	19.2	7.7	23.1	23.1	11.5	11.5	3.9
Lack of patient and family interest in LTFU activities	**%**	19.2	15.4	11.5	26.9	7.7	19.2	0.0
Lack of NBS program interest in LTFU activities	**%**	38.5	15.4	3.9	19.2	7.7	3.9	11.5

*You may select "N/A" if the listed item is NOT AT ALL a barrier for your program.

GENERAL ATTITUDES

Does your program consider these LTFU activities to be part of its responsibilities?

		Collect LTFU Data	
		No	Yes
LTFU part of program activity?	Yes	16.7%	57.7%
	No	50.0%	30.8%
	Maybe	33.3%	11.5%

Who does your program believe should be responsible for carrying out these LTFU activities?

Common Responses

- Specialists
- Medical home
- Children with special healthcare needs program

How feasible will it be for your program to collect LTFU data for these categories within the next five years?

For states that DO NOT collect LTFU data:

		Less Feasible					More Feasible	
		1	2	3	4	5	6	Total n
Patient Demographics	%	33.3	8.3	25.0	8.3	8.3	16.7	12
Health Care Utilization	%	41.7	16.7	16.7	8.3	0.0	16.7	12
Treatment Regimen	%	41.7	8.3	25.0	25.0	0.0	0.0	12
Health Outcomes	%	41.7	8.3	33.3	16.7	0.0	0.0	12
Follow-Up Status	%	41.7	8.3	16.7	25.0	0.0	8.3	12
Cost	%	75.0	16.7	0.0	8.3	0.0	0.0	12
Patient Access to Services	%	41.7	16.7	16.7	8.3	0.0	16.7	12
Enrollment in Research Studies	%	41.7	25.0	16.7	16.7	0.0	0.0	12

Your program does not collect LTFU data on the categories in the table below. How feasible will it be for your program to collect LTFU data for these categories within the next five years?

For states that DO collect LTFU data:

		Less Feasible					More Feasible	
		1	**2**	**3**	**4**	**5**	**6**	**Total n**
Patient Demographics	%	0.0	66.7	0.0	0.0	0.0	33.3	3
Health Care Utilization	%	20.0	60.0	20.0	0.0	0.0	0.0	5
Treatment Regimen	%	16.7	33.3	33.3	16.7	0.0	0.0	6
Health Outcomes	%	22.2	22.2	11.1	33.4	0.0	11.1	9
Follow-Up Status	%	0.0	100.0	0.0	0.0	0.0	0.0	1
Cost	%	50.0	22.7	18.2	0.0	0.0	9.1	22
Patient Access to Services	%	31.3	18.8	6.2	25.0	6.2	12.5	16
Enrollment in Research Studies	%	50.0	30.8	15.4	3.8	0.0	0.0	26

CONCLUSIONS

- About 2/3 of states gather LTFU data with variation in the type of data collected
- Most of these collect it themselves (as opposed to access existing data or contract out collection)
- Most have not used the SACHDNC statement to guide development of LTFU collection
  - those that have are more likely to gather LTFU
- Frequently cited barriers to collection among states not collecting include data sharing regulations, lack of statutory authority, lack of designated employee

IMPLICATIONS

- Barriers to LTFU collection are multifaceted and include policy, resources and values
- States may need guidance regarding LTFU data collection
  - Prioritization of data collection type
  - Goal of LTFU data collection
    - programmatic evaluation vs. clinical care
- LTFU issues become increasingly important as disorders with late-onset phenotype are added to the RUSP

ACKNOWLEDGEMENTS

- State Programs for their participation
- UM Research Team Members:
  - Caitlin Finan
  - Anne Azrak
- Collaborators:
  - Aaron Goldenberg, PhD (Case Western)
  - Jelili Ojodu (APHL)
- Funding from HRSA & Genetic Alliance