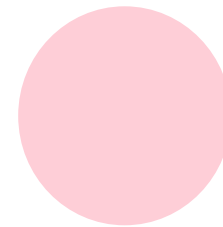
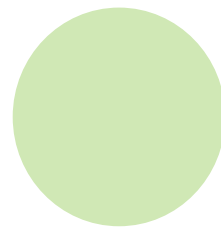
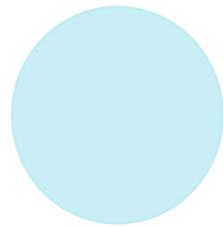


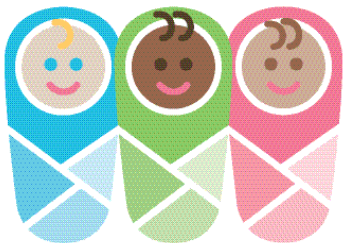
The “Value of Values”:
Expanding Assessment of Net
Benefits and Harms through
Social Science Data



Aaron Goldenberg, PhD MPH

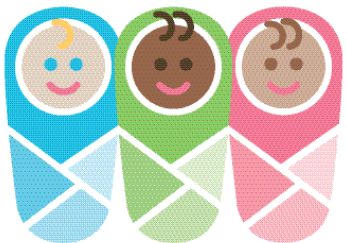
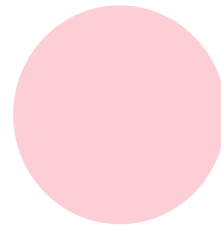
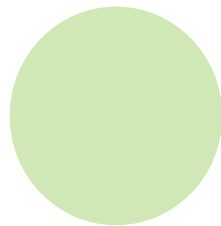
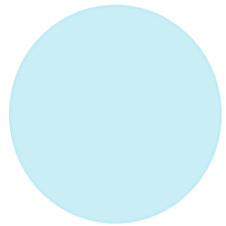
Department of Bioethics

Case western Reserve University School of Medicine



Disclosures

I have nothing to disclose



Evolving NBS Needs an Evolved Evidence Base

Why social science research in NBS is so crucial

- ❖ We are on the cusp of a transformation in NBS practices and research
 - Expansion of NBS Panels (later onset, potentially more uncertainty)
 - Use of Genomic Screening
 - Storage and Uses of Residual DBS
- ❖ However, 1) there is a dearth of data pertaining to public and parental values related to these changes 2) what data exists is underutilized
- ❖ Vital to have data from key stakeholders to help manage expansion in a transparent manner and maintain the ethical justification of NBS
- ❖ These kinds of data are also critical for expanding notions of benefits and harms to include individual newborns, families, and society



Newborn screening program used to aid criminal investigation, public defender says
Advocates alarmed over potential breach of Fourth Amendment protections
BY NIKITA BIRYUKOV • JULY 12, 2022 2:44 PM



MAINTAINING
TRUST
IN
NEWBORN SCREENING

COMPLIANCE AND INFORMED CONSENT
IN THE NETHERLANDS

BY SPOON VAN DER BURG AND MARCEL VERWEJ

What do we mean by assessing “values”?

Whose Values (Perspectives, Concerns, Exceptions)?

- Parents with children with the condition
- Parents generally
- General Public

Values about what?

- Condition Specific Issues vs. General NBS issues
- NBS disparities and access to care

When?

- During the pilot stage
- In states that are already screening
- As an activity of the committee during evidence review

How?

- Surveys/Interviews with individuals/families
- Focus groups or small group dialogue
- Deliberative democracy or other innovative approaches

Why do this?

- Evidence Review/Decision Matrix Process: To impact the final “score” and Committee’s yes/no decision
- Evidence Review Process: To impact the committee's recommendation (ie state resources for parents and clinicians, choices of variants, return of results)
- Recommendations for resources? Access? Follow up? Education? Consent for some conditions?

Problematising Current Approaches

Challenge 1

- NBS harms or benefits to families/society raised are either anecdotal or hypothetical
- Burden of proof has historically been higher for benefits
- ***Problem:*** Lack of data or data is not known

Challenge 2

- Data on NBS harms or benefits to families/society is dismissed as anecdotal or not “scientific”
- ***Problem:*** Lack of understanding regarding social science data

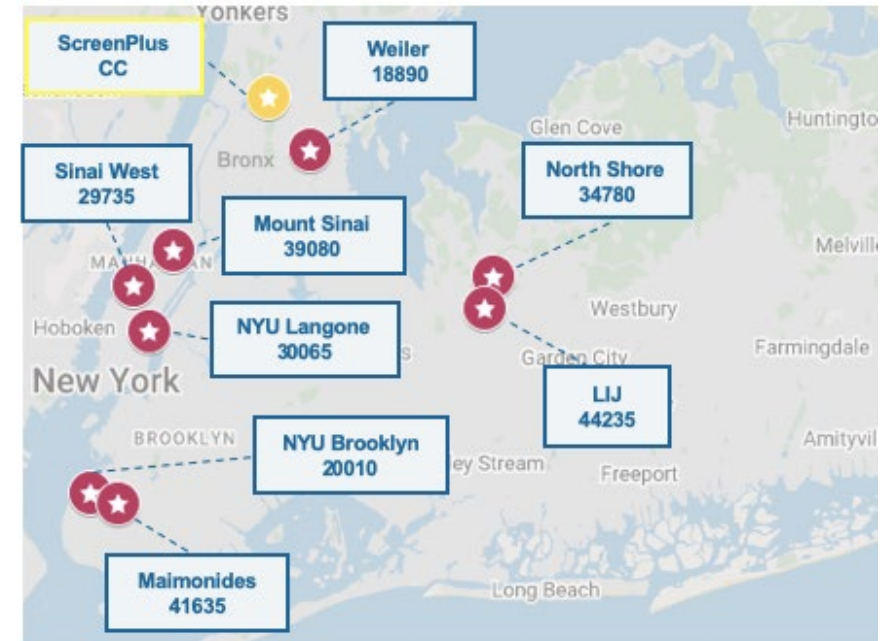
Challenge 3

- Data on harms or benefits to family/society is not systematically integrated into evidence review
- ***Problem:*** Social science data does not fit within traditional matrix processes

Example: ScreenPlus

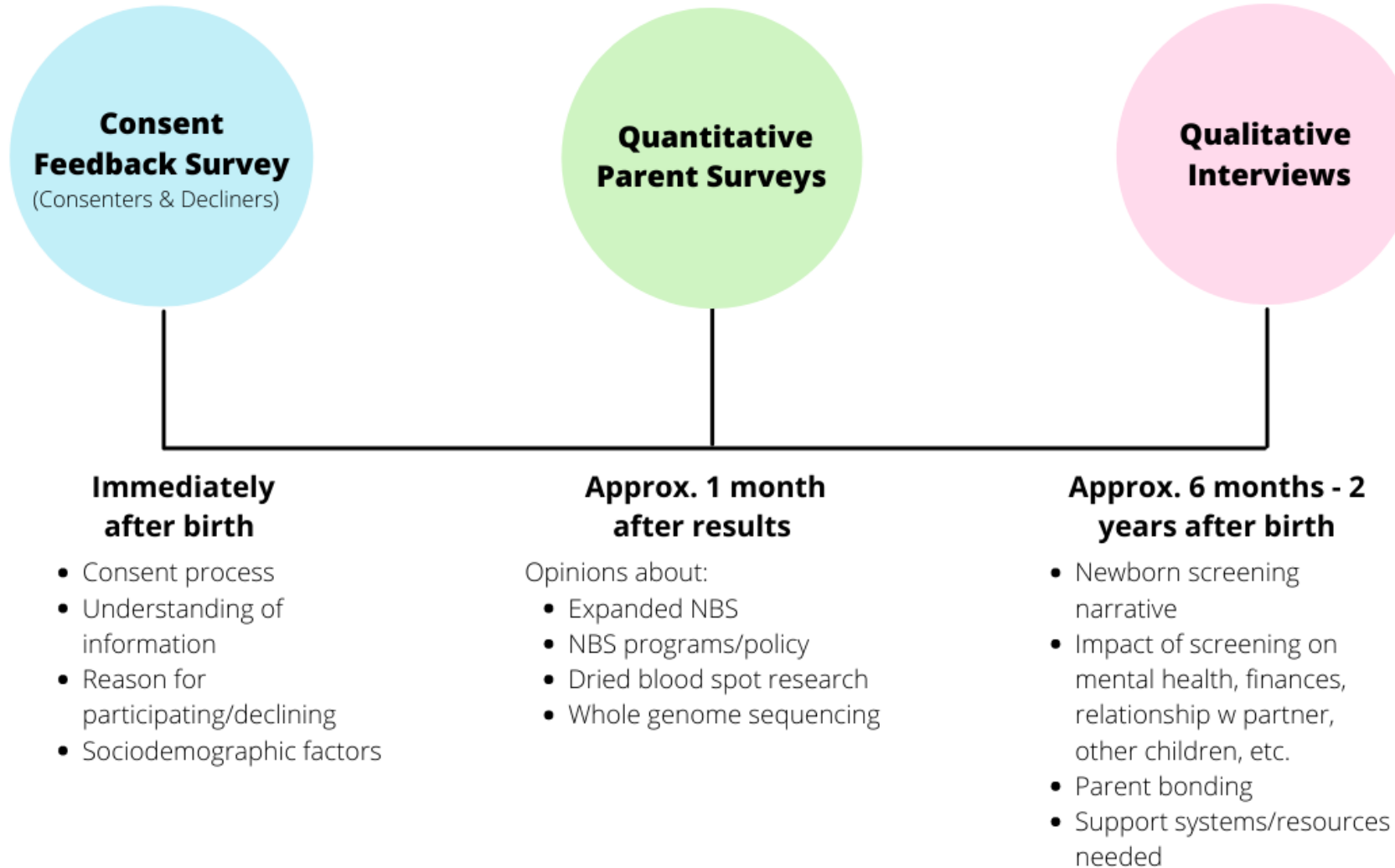
A comprehensive, flexible, multi-disorder newborn screening program

- ❖ Consented pilot NBS run in conjunction with the NYS Newborn Screening Program.
- ❖ Enroll 100,000 babies born at 8 high birthrate, ethnically diverse hospitals over five years.
- ❖ Goal to assess the analytic and clinical validity of multi-tiered screening for a fluid panel of disorders
- ❖ Assess ethical, legal, and social issues from parental feedback surveys and interviews with parents of children



Example: ScreenPlus

Ethical, Legal, and Social Implication Studies: Parental Perspectives



ScreenPlus: Survey Themes

Consent Survey

What was most helpful?

Main reasons for participating?

Dried Blood Spot Retention

Parental consent: if and when

Feelings about specific uses

Decliner Survey

Main reasons to not participate?

Re-defining the Benefits of NBS

Reproductive planning?

Future planning?

At risk relatives?

Diagnostic odyssey?

Newborn Screening Expansion

What types of disorders should be included?

- Age of onset
- Treatability
- Diagnostic and prognostic uncertainty

Newborn Sequencing

Trust in government and commercial entities

Data privacy concerns

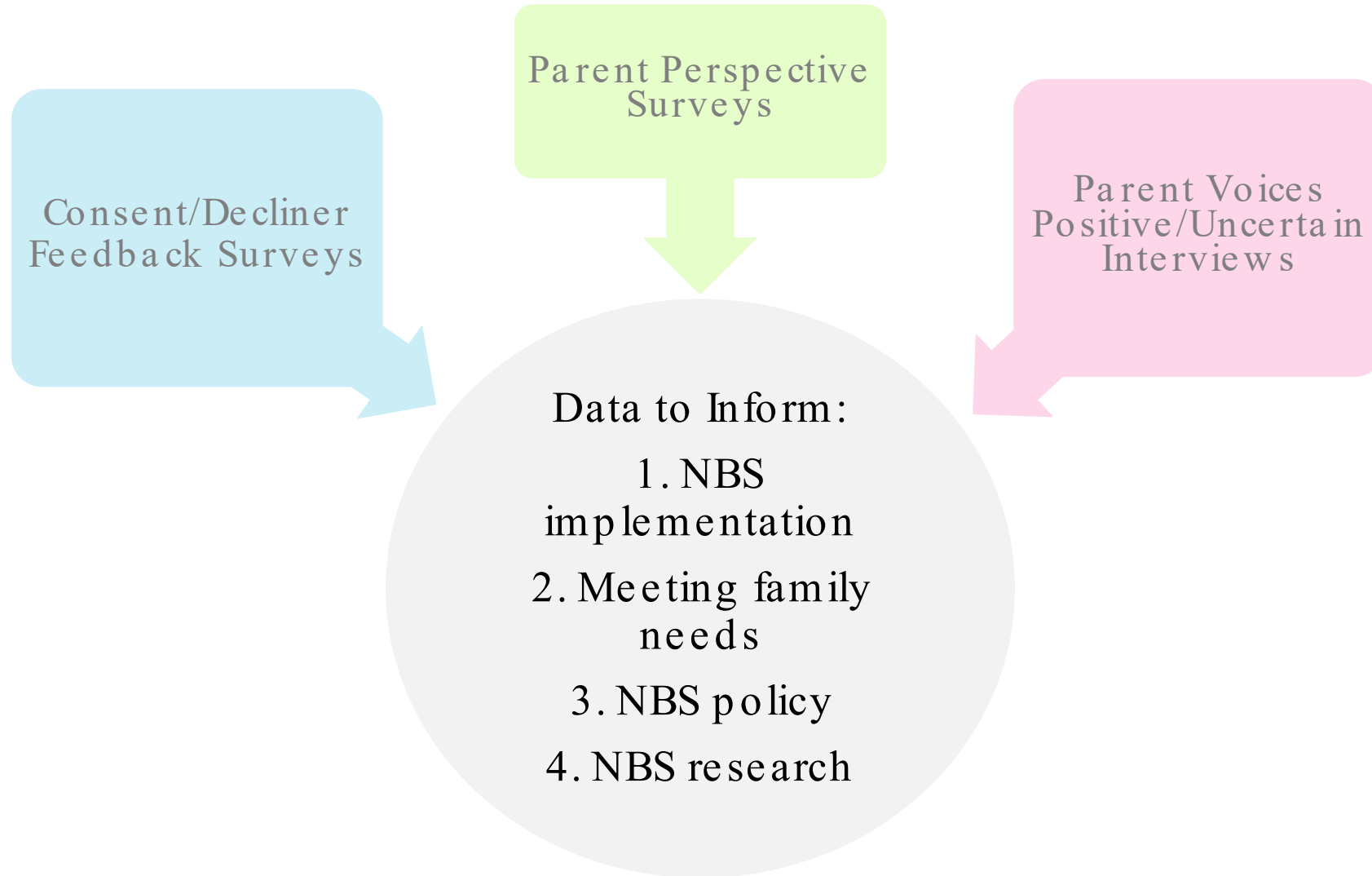
Equity and diversity

What information should be returned?

- Actionable vs non-actionable?
- Known P/LP vs VUS?
- Genetic risks?

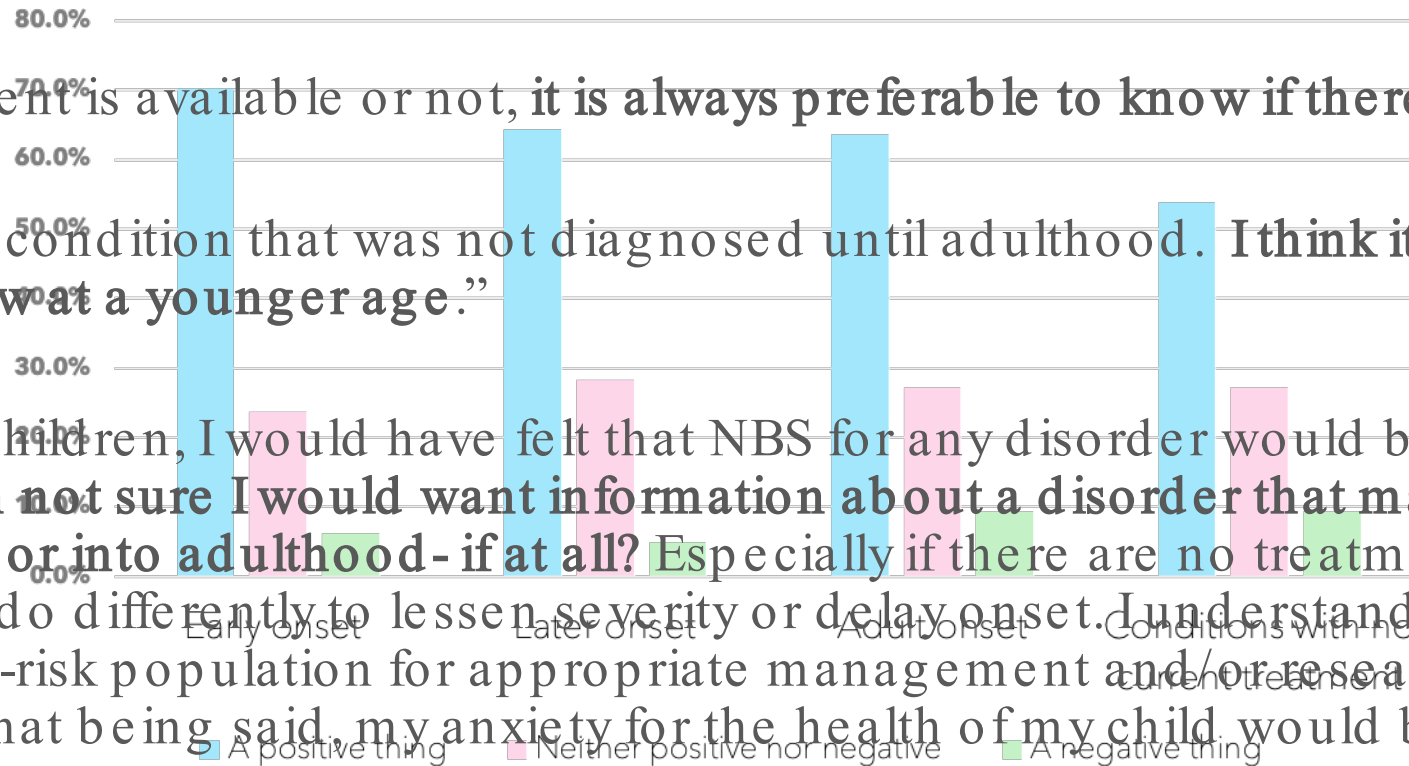
All surveys capture demographic data including parental age, socioeconomic status, ancestry, general views about religiosity and politics

Data Goals



Need for Both Qualitative and Quantitative Data

Parental Attitudes Towards NBS (N=225)



- “Whether treatment is available or not, it is always preferable to know if there is an issue.”
- “I have a genetic condition that was not diagnosed until adulthood. I think it would have been very beneficial to know at a younger age.”
- “Prior to having children, I would have felt that NBS for any disorder would be a positive. Now that I have a child, I am not sure I would want information about a disorder that may not affect my child for several years, or into adulthood- if at all? Especially if there are no treatments, or currently anything I could do differently to lessen severity or delay onset. I understand the importance of identifying the at-risk population for appropriate management and/or research for potential treatments, but that being said, my anxiety for the health of my child would be shook, knowing future risks.”

Context, Context, Context...Uncertainty

- ❖ Parents asked about their interest in having their child screened for a variety of conditions.
- ❖ Agree or disagree with the following statements...
 - ❖ I would like to get my baby's newborn screening results...

In cases where my baby is at high risk to develop a serious health condition that might need treatment, but doctors cannot tell me when they will get sick (N=286)

Strongly/Somewhat Agree ---92%

Strongly/Somewhat Disagree ---8%

In cases where doctors cannot tell me if my baby really has a serious condition (N=286)

Strongly/Somewhat Agree ---70%

Strongly/Somewhat Disagree ---30%

Context...what population/community?

- ❖ Agree or disagree with the following statements...
 - ❖ I would like to get my baby's newborn screening results...

In cases where doctors cannot tell me if my baby really has a serious condition (White non-Hispanic)
N= 122

Strongly/Somewhat Agree ---60%

Strongly/Somewhat Disagree ---40%

In cases where doctors cannot tell me if my baby really has a serious condition (Non-white)
N=145

Strongly/Somewhat Agree ---80%

Strongly/Somewhat Disagree ---20%

Context...how are the questions asked?

Survey 1	Survey 2
I would like to get <u>my baby's</u> newborn screening results...	<u>All babies</u> should receive screening results...
In cases where doctors cannot tell me if my baby really has a serious condition N= 286	In cases where doctors cannot tell me if my baby really has a serious condition N= 240
Strongly/Somewhat Agree---70%	All babies should be be screeded (mandatory)...50%
Strongly/Somewhat Disagree---30%	Only babies whose parents give permission...50%

Context...quantitative vs. qualitative

In cases where doctors cannot tell me if my baby really has a serious condition
N= 286

Strongly/Somewhat Agree ---70%

Strongly/Somewhat Disagree ---30%

“The only thing I would hate to add to a new mom is additional worry. If there is any uncertainty about the serious condition or no possible treatment, it's honestly better to live in ignorance and enjoy your baby vs always being worried one day they might get sick. Please don't cause any unnecessary anxiety for parents”

“Multiple doctors visits early in a baby's life are very stressful. Knowing that might be coming, or that there is a diagnosis, would be valuable to help manage uncertainty.”

“The more information we have, the better. There are so many things that we don't know and can't predict about our own bodies, and having the opportunity to know more about my baby's health and probabilities is comforting”

“I worry that, newborns may be administered unnecessary treatments on the basis of potentially having a condition. While I agree, it is important to catch things early in order to treat them properly. It is just as important to be diligent and rule out potential with reality. Just because something can potentially happen doesn't necessarily mean that it will and I don't think it is conducive to treat somebody for something they may or may not have.”

Moving forward...together

Challenge 1

- Data anecdotal or hypothetical



- “Co-create” research questions
- Develop research that include ELSI and social science research methods
- Funding!

Challenge 2

- Data dismissed as anecdotal or not “scientific”



- Create new opportunities for presenting/integrating social science data
- Develop training opportunities in NBS programs to work with social science data

Challenge 3

- Data not systematically integrated into evidence review



- Further develop decision matrix to integrate “value data” more comprehensively
- These data may not help “decide” (but could inform post screening needs)

Addressing Challenge 3:

Integrating values/perspectives into a decision matrix

- ❖ ELSI and Social Science data will help but,
 - ❖ There will always be divergent and pluralistic views on NBS issues
 - ❖ But that's not a failure of the data...it's the reality

- ❖ We must find better ways to address this?

Determine
Thresholds of
Potential Harms

Weigh Screening
vs. Clinical Harms

“Value” data may
indicate other
resource/system
needs

Consider when
permission/consent
may be necessary

Promoting the Value of Social Science in Policy

Arthur Lupia, Political Scientist (University of Michigan)

Core Principles:

- ❖ Rigor (how we know what we know)
- ❖ Ethics and Ethical Research
- ❖ Precision in measurement and conceptualization
- ❖ Causality

Lupia A. What Is the Value of Social Science? Challenges for Researchers and Government Funders. *PS: Political Science & Politics*. 2014;47(1):1-7.

<https://cpsblog.isr.umich.edu/?p=2893>

Let's not *totally* reinvent the wheel...

❖ Connect with scholars and scholarship in our own community
696 Matern Child Health J (2016) 20:693–700

Table 1 Potential harms associated with newborn screening

Aspect of newborn screening	Type of potential harm	
	Newborns	Parents/families
Screening (bloodspot or point-of-care)	Pain or other adverse impacts from screening False positives or false negatives of screening	Psychosocial harms associated with false positive laboratory results for unaffected infants
Diagnosis evaluation	Pain or other adverse impacts from diagnostic testing Missed or incorrect diagnosis Disparities in access to diagnostic testing ^a	Psychosocial harm from diagnostic or prognostic uncertainty in diagnosis, or degree or age of onset of disease manifestations
Treatment and long term follow-up	Pain or other adverse impacts of treatment Treatment with an uncertain impact of disease severity and/or the timing of manifestations Disparities in access to treatment ^a	Psychosocial harm from uncertainty of outcomes Psychosocial, financial or other harms associated with long-term treatment Psychosocial harm from treatment decisions

^a Such disparities could be considered a harm if disparities associated with screening were more pronounced than those encountered with clinical presentation

Acknowledgements

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