An Assessment of Public Preferences for Newborn Screening Using Best–Worst Scaling

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Objective To identify and quantify public preferences for attributes of newborn screening conditions.

Study design We conducted an online national survey of the public (n = 502) to evaluate preferences for attributes of candidate newborn screening conditions. Respondents were presented with hypothetical condition profiles that were defined using 10 attributes with 2-6 levels per attribute. Participants indicated whether they would recommend screening for a condition and which condition attributes were most and least important when making this decision (best–worst scaling). Difference scores were calculated and stratified by condition recommendation (recommend or not recommend for screening). Regression analyses were used to evaluate the effect of attributes on choice to screen or not screen.

Results The number of babies diagnosed was important to those who would recommend newborn screening for a profile, and age at which the treatment would start was important to those who would not recommend newborn screening. Cost was considered to be a key attribute, and treatment effectiveness and impact of making the diagnosis through newborn screening were of low importance for both groups.

Conclusion Public preferences identified through survey methods that provide an adequate baseline understanding of newborn screening can be used to inform newborn screening decisions. *(J Pediatr 2018;■■:■■-■■).*

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Newborn screening is one of the most successful public health programs, having screened hundreds of millions of children for serious inherited disorders and saved many newborns from death and severe disability.[1] The decision about which conditions to add to a state newborn screening panel can be made through a number of processes, such as legislative mandate, department of public health regulations, or vote by a committee of medical and public health experts. The decisions also may be influenced by the Advisory Committee on Heritable Disorders in Newborns and Children, which provides evidence-based recommendations about which conditions should be added to a Recommended Uniform Screening Panel for all states.[2]

Public input in decisions about which disorders should be screened through newborn screening has been limited to public advocacy. This important, but select, view of public preferences[3] often is provided by parents of affected children or advocates for rare disease organizations. Missing is meaningful input from the general public, who finance the programs through tax dollars and whose children undergo this mandatory screening. The view of the general public is important because there are limited resources available to implement screening for the increasing numbers of candidate disorders. As a result, decisions must be made about which disorders should be given preference for screening based on a number of disorder characteristics (eg, number of children diagnosed, success of treatment). When available evidence must be placed in a value context, the public's preference regarding newborn screening conditions can provide useful additional information for newborn screening programs.

A number of US- and Canadian-based studies have attempted to examine the public's preferences about newborn screening.[4-7] However, these studies suffer from limitations that affect generalizability of their findings. First, these studies frequently queried public opinion about specific conditions—as opposed to the specific attributes, or characteristics (eg, age of onset, success of treatment), of conditions. Queries about conditions, rather than disease characteristics, may bias respondents based on their previous experience with the condition. Second, some studies were conducted by using focus groups and/or general surveys of select populations and did not have a broad and diverse representation of the general public.

Understanding which attributes, or characteristics, of candidate newborn screening conditions are important to the general public may be useful information for newborn screening program committees to consider alongside their current

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Funded by Agency for Healthcare Research & Quality (AHRQ) (R01HS020644). Study sponsors did not have a role in the study design; collection, analysis, and interpretation of the data; the writing of the report; or the decision to submit this manuscript for publication. The authors declare no conflicts of interest.

Portions of this study were presented at the Pediatric Academic Societies annual meeting, April 30-May 3, 2016, Baltimore, Maryland.

0022-3476/$ - see front matter. © 2018 Published by Elsevier Inc.
https://doi.org10.1016/j.jpeds.2018.05.035

evidence-based evaluation—especially when evidence is limited, as is often the case with rare disorders.[8,9] Given the paucity of information concerning US public preferences about candidate newborn screening conditions for this mandatory public health screening program, we aimed to identify and quantify public preferences for important condition attributes or characteristics.

Methods

An online survey was used to quantitatively measure the relative value of attributes for newborn screening candidate conditions using best–worst scaling. In this study, we used best–worst scaling, a preference measurement method from marketing economics and type of conjoint analysis.[10] Best–worst scaling provides the relative value of characteristics of a condition (eg, age of onset, success of treatment) by asking the respondent to choose the best and worst attribute from among a list. Best–worst scaling has been used in healthcare to identify characteristics that are most important to healthcare decision-making[11,12] and research policymaking[13] and to better understand the most and least important factors associated with health programs.[14] This study used a profile case approach as described by Flynn et al[12]. A set of attributes was identified to describe candidate conditions for newborn screening. Each attribute was associated with a set of levels to describe the possible states of that specific attribute (eg, the attribute for age of onset would include levels of infancy, childhood, and adulthood) (**Table I**). Respondents are asked to review a profile (hypothetical condition) and to select the most and the least important factors (attribute/level) in the profile.[15] By requesting 2 responses, more data about the respondent's preferences can be collected, providing greater insight into the respondent's decision-making process.[15]

Eligible participants were sampled randomly from GfK KnowledgePanel, a nationally representative online panel of US adults that has been widely used in other pediatric health surveys.[16-18] Eligible participants were noninstitutionalized adults age 18 years and older residing in the US and were surveyed from September to October 2015. This study was approved by

Table I. Condition attributes and levels

Attributes	Levels
1. Number of babies diagnosed	1. 1 in 100 000 2. 5 in 100 000 3. 10 in 100 000 4. 20 in 100 000
2. Chance that a positive newborn screening test result is wrong	1. 1% 2. 7% 3. 28% 4. 80%
3. Cost of confirming testing and diagnosis	1. $10 2. $100 3. $1000 4. $10 000
4. Likelihood of developing symptoms	1. Very unlikely to develop symptoms 2. Unlikely to develop symptoms 3. Likely to develop symptoms 4. Very likely to develop symptoms
5. Seriousness of symptoms without treatment	1. A child would be able to do all daily activities 2. A child would be able to do most daily activities 3. A child would be able to do some daily activities 4. A child would be able to do no daily activities
6. Age when symptoms develop and life expectancy without treatment	1. Symptoms develop in infancy; child expected to die in infancy 2. Symptoms develop in infancy; child expected to die in childhood 3. Symptoms develop in childhood; child expected to die in childhood 4. Symptoms develop in childhood; child expected to die in adolescence 5. Symptoms develop in childhood; child expected to die in adulthood 6. Symptoms develop in adulthood; child expected to die in adulthood
7. Start of treatment	1. Within a few weeks of birth 2. During the first year of life 3. During childhood 4. During adolescence
8. Success of treatment	1. Treatment will cure the disease 2. Treatment will prevent the disease from getting worse 3. Treatment will slow the worsening of the disease 4. No treatment is available, but care will be given to relieve symptoms
9. Side effects of treatment	1. Potential death 2. Serious side effects of treatment, but will not die 3. Mild side effects 4. None
10. Impact of diagnosis through newborn screening	1. Parents will know about the baby's disease sooner and treatment will be more successful 2. Parents will know about the baby's disease sooner, but this will have no change on the success of treatment

FLA 5.5.0 DTD ■ YMPD10020_proof ■ July 13, 2018