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Original Article

Willingness to Pay for a Newborn Screening Test for Spinal Muscular Atrophy

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ABSTRACT

OBJECTIVES: The current US mandatory newborn screening panel does not include spinal muscular atrophy, the most common fatal genetic disease among children. We assessed population preferences for newborn screening for spinal muscular atrophy, and how test preferences varied depending on immediate treatment implications. **METHODS:** We conducted an online willingness-to-pay survey of US adults (n = 982). Respondents were asked to imagine being parents of a newborn. Each respondent was presented with two hypothetical scenarios following the spinal muscular atrophy screening test: current standard of care (no treatment available) and one of three randomly assigned scenarios (new treatment available to improve functioning, survival, or both). We used a bidding game to elicit willingness to pay for the spinal muscular atrophy test, and performed a two-part model to estimate median and mean willingness-to-pay values. **RESULTS:** Most respondents (79% to 87%) would prefer screening their newborns for spinal muscular atrophy. People expressed a willingness to pay for spinal muscular atrophy screening even without an available therapy (median: $142; mean: $253). Willingness to pay increased with treatment availability (median: $161 to $182; mean: $270 to $297) and respondent income. Most respondents considered test accuracy, treatment availability, and treatment effectiveness very important or important factors in deciding willingness to pay. **CONCLUSIONS:** Most people would prefer and would be willing to pay for testing their newborn for spinal muscular atrophy, even in the absence of direct treatment. People perceive the spinal muscular atrophy test more valuable if treatment were available to improve the newborn's functioning and survival. Despite preferences for the test information, adding spinal muscular atrophy to newborn screening programs remains controversial. Future studies are needed to determine how early detection may impact long-term patient outcomes.

Keywords: spinal muscular atrophy, willingness to pay, newborn screening, preferences

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Introduction

Newborn screening identifies potentially fatal and disabling conditions in infants shortly after birth. Early diagnosis can provide a window of opportunity for early intervention to prevent premature death or the need for long-term care.[1] Every year, over four million US newborns are screened for various congenital disorders, such as genetic and metabolic disorders, hormonal disorders, and hearing loss. Traditionally, newborn screening programs have included screening tests for disorders that have a clear advantage of early diagnosis, especially those with a readily available treatment of proven efficacy.[2] However, there is a

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growing emphasis on the broader benefits of newborn screening, even for diseases without available treatment. For example, test information could aid health care decision making, even if the test results do not have treatment implications or affect clinical management.[3,4] In addition, effective screening could help families avoid a long, expensive "diagnostic odyssey" during which unnecessary testing is done in the attempt of diagnosing an affected patient.[5] Screening tests also may allow for preparation and early palliative care and provide an opportunity for affected children to participate in clinical trials for new interventions.[2] Furthermore, test information may be valuable for nonmedical reasons, such as family and financial planning, as well as "knowing for knowing's sake" to reduce uncertainty.[6,7]

Spinal muscular atrophy (SMA) is the most common, fatal genetic disease among children, affecting approximately 1 in 10,000 live births.[8] SMA is an autosomal recessive neuromuscular disorder characterized by progressive muscle weakness, respiratory problems, and difficulties with basic activities of daily living (e.g., eating and moving).[9] Depending on the SMA type, health outcomes range from early infant death to normal adult life with mild symptoms.[9] There is currently no effective long-term treatment regimen for the disease,[2] although interventions exist to manage the symptoms and prevent complications.[10]

Despite the seriousness of the condition, none of the current mandatory newborn screening programs in the United States include SMA testing. The SMA test would require a blood sample from the infant, and it is nearly 100% accurate in detecting the disease.[9,11,12] There have been many discussions among physicians, policymakers, and patient advocacy groups with regard to screening newborns for SMA.[1,2,5,10] The screening test may identify presymptomatic newborns who may be eligible for participating in clinical trials for potential therapies for SMA.[11] Should direct treatment become available, newborns with SMA may initiate therapy before the degeneration of motor neurons.[13]

The objective of this study was to understand the general public's preferences for SMA newborn screening. We conducted a US population-based willingness-to-pay (WTP) survey to investigate the perceived value of a newborn SMA screening test. The WTP methods have been used to estimate the value of a wide range of health care interventions, including diagnostic tests,[14] and have been used extensively across different disease areas.[15,16] In this study, we assessed whether and how much people would pay for the newborn SMA test. We also examined how preferences varied depending on whether the test information had any immediate treatment implications.

Methods

Survey design

We presented respondents with hypothetical scenarios in which they were asked to imagine that they were the parent of a newborn. The survey included a description of SMA symptoms, prevalence rate, and testing information (e.g., test procedure and accuracy). Respondents were given the option of testing their newborn for SMA; however, they would have to pay for the test themselves, because the health insurance did not cover it. The survey incorporated four sets of hypothetical scenarios, each of which described a different treatment option following the SMA test. Each respondent was presented with two sets of scenarios: the baseline scenario and one of three alternative treatment scenarios that were randomly assigned.

The baseline scenario described the current standard of care (SOC), stating that there is no cure or medical treatment available to prevent SMA. It also stated that the SOC involved managing the symptoms and preventing complications due to difficulties eating, breathing, and moving. We also developed three alternative scenarios in which treatment for SMA was available:

(1) Improved functioning: New treatment may improve the newborn's daily functioning, growth, and physical development, but it does not affect how long the newborn would live;
(2) Improved survival: New treatment may allow the newborn to live longer than with current standard care, but it would not improve functioning, growth, or physical development; and
(3) Improved functioning and survival: New treatment would improve both functioning and survival of the newborn.

The WTP approach allows researchers to extract estimates of the willingness of individuals to pay for the health care intervention under consideration.[15] Following recommended practice for WTP research, we used a double-bounded, dichotomous-choice approach, which presented respondents with a binary (yes/no) bidding game, to elicit respondents' perceived value for the test.[14] This approach presented respondents with a series of prices and asked them if they would be willing to pay at least that amount for the test. The initial bid was $50 in all scenarios. We chose this initial value because it reflects typical out-of-pocket costs for a newborn blood test. Respondents answering "yes" to the first question were asked if they would pay twice the initial price ($100). Respondents answering "no" were asked if they would pay half the initial price ($25). If the respondent rejected both bids, the subject was asked if they would test the newborn if the test were free. For respondents answering "no" to this question, the sequence of questions ended. For all other respondents, an open-ended question asked them to indicate the maximum amount (in dollars) they would pay for the test.

Additionally, we elicited information on respondents' demographic characteristics and socioeconomic status. We also asked respondents to rate the importance of several possible factors in their decision for the SMA test and to provide any other factors important to their decisions. The survey instrument is available from the authors upon request.

Survey administration

The questionnaire was administered via the Internet to a national panel of US adults, 18 years of age and older, maintained by a survey research firm, GfK Knowledge Networks (CA, USA). The panel recruitment methodology and sampling frames have been described in detail elsewhere.[17] Briefly, panel members were randomly recruited by telephone, self-administered mail, and web surveys, using a probability sampling frame to provide a nationally representative sample that covered both the online and offline populations in the United States. Households were provided with access to the Internet and hardware if needed to minimize self-selection bias.

We pilot-tested the questionnaire with 35 subjects to assess whether questions were clear and logical, and whether respondents could comprehend them. The online survey was sent to 1879 adults in the GfK Knowledge Networks panel, and 1023 agreed to participate (response rate: 54%). Of these, 1010 completed both the baseline and the treatment scenario. We removed 28 individuals who provided invalid responses to at least one of the two scenarios they received. In these invalid responses, their open-ended WTP value did not fall into the range of WTP given by the bidding game (e.g., a respondent who answered "no" to an initial bid of $50 and "yes" to a second bid of $25 but did not provide an open response value between $25 and $50). Our final analytic sample included 1964 responses from 982 individuals (Fig 1). In sensitivity analysis, we examined an expanded sample that included respondents who provided valid answers to only one of their assigned scenarios. This expanded sample consisted of 1992 responses from 1010 respondents.