Caregiver Burden in Fragile X Syndrome as a Function of a Health-State Utility Index

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Background
- Fragile X syndrome (FXS), the most common inherited form of intellectual disability, causes moderate to severe intellectual delays in males and milder, yet significant, limitations in females.
- The incidence of FXS in the United States (US) is approximately 1:4,000 males and 1:4,000-6,000 females.1
- Previous research suggests that parents of a child with FXS confer risk in such domains as stress,2 depression and anxiety,3 family income4 and parenting5 and employment6 outcomes for parents of children and adults with FXS.
- Although research has shown that caregivers of patients with special health care needs experience elevated burden,7 few data focus specifically on the burden of care for children and adults with FXS.
- The Aberrant Behavior Checklist-Community Version (ABC-C) is a parent-completed measure of a child’s behavior across a number of maladaptive domains.8 Recently, a utility index, the ABC-C, was derived from the ABC-C to reflect health-related quality of life (HRQOL) changes in patients.

Objective
- To assess the relationship between health care resource utilization by patients with FXS, as well as burden experienced by their caregivers, with the ABC-C UI

Study Design
- A retrospective analysis of results from a 2011 survey of US caregivers of children and adults with FXS.11
- Invitations to participate in this survey were sent to 508 families of children (>5 years of age) and adults with FXS enrolled in a research registry.
- Survey questions collected information on standardized measures of FXS-related behavior, attention, and anxiety problems, as well as resource-use parameters

Study Measures
- Demographic characteristics of patients with FXS (i.e., age, sex, family income)
- Overall cognitive ability of individuals with FXS (i.e., poor, fair, good, very good)
- Resource use by individual with FXS (i.e., visits to medical specialists and medical tests performed in the past year, and prescription medications currently used)
- Caregiving requirements (number of hours of care they or other family members provided during a typical day for FXS-related behavioral, attention, anxiety, or physical care)
- Economic and employment impact (extent to which FXS caused a financial burden or family member to quit working)
- Caregiver injuries (injuries caused by individual with FXS in the past year)
- Caregiver mental health (whether caregiver had been treated by a professional for depression, stress, or anxiety in the past year)
- The ABC-C (7 items with 3 response options each, reporting utilities from 0 = dead to 1 = full health); five utility score values were significantly more likely to have had ≥5 (versus <5) specialist visits in the past year (odds ratio = 10.2; P = 0.0030), more frequent caregiver injuries (β = –7.540; P = 0.0043), and high (0.78–0.89), and very high (0.90–1.00) ABC-UI score category, 31% low, 27% moderate, 38% high, and 3% very high

Analytic Approach
- Descriptive analysis and summarized characteristics of the study population
- Multinatural logistic regression models formally assessed the association between ABC-C score and 9 FXS burden-relevant outcomes controlling for sex, age, overall cognition ability, and family income:
- The likelihood of ≥5 (versus ≤4) specialist visits in the past year
- The likelihood of ≥0 (versus ≥1) medical tests or procedures in the past year
- The likelihood of ≥20 (versus ≥10) prescription medications currently used
- The likelihood of ≥8 (versus ≥4) hours, on average, of daily unpaid caregiving
- The likelihood of <5 (versus ≥5) hours, on average, of daily paid caregiving
- The likelihood of ≥1 (versus ≤0) caregiver injuries related to FXS-related caregiving in the past year
- The likelihood of ≥1 (versus ≤0) mental health-related visits related to FXS-related caregiving in the past year
- The likelihood of high (versus low) financial burden
- The likelihood that a family member did (versus did not) ever quit working due to FXS-related caregiving
- Separate models were estimated for each outcome which included continuous ABC-C score as the main independent variable of interest
- Pretreatment ABC-C scores were replaced using the continuous utility index score with the 5 ABC-C score categories that were developed.
- The study was approved by the RTI International Institutional Review Board

Results

Responder Characteristics
- 310 caregivers agreed to participate in the survey, and 340 provided complete information necessary to calculate the ABC-C UI score
- Further description of responder characteristics have been published11

Patient Characteristics (Table 1)
- Individuals with FXS were approximately 10 years of age, 83% were male, 1% were white, and 50% were classified as having “fair” cognitive ability
- The mean [SD] ABC-C score was 0.7 (0.2), and 2% of individuals with FXS were in the very low ABC-C score category, 31%, 27%, moderate, 38%, high, and 3% very high

Burden of Illness (Table 2)
- In the past year:
- The mean [SD] number of medical tests or procedures performed on an individual with FXS was 1.1 (0.5), and 45% used 2 prescription medications
- On average:
- The mean [SD] number of hours of unpaid caregiving was 8.4 (5.6), and 32% reported ≥8 hours of unpaid caregiving
- The mean [SD] number of hours of paid caregiving was 0.7 (0.4), and 53% reported earning ≥0 hours of paid caregiving
- 57.2% of caregivers reported that FXS caregiving imposed a high financial burden on their family, and 37.7% reported that a family member quit working to provide FXS caregiving

Multivariable Regression Model Results: Continuous ABC-C UI Score
- Caregivers whose children had higher ABC-C scores were less likely to experience high financial burden (P = 0.097; P = 0.0205)
- Individuals with lower ABC-C scores had more visits to specialists (β = 4.58, P = 0.0001) and used more prescription medications (β = –4.57; P = 0.0001)
- Caregivers of individuals with lower ABC-C scores experienced greater burden, including more hours of unpaid caregiving (β = –2.18; P = 0.0023), more frequent caregiver injuries (β = 0.54; P = 0.0001), and more caregiver visits to mental health professionals (β = –0.62; P = 0.0001)

Multivariable Regression Model Results: ABC-C UI Score Categories
- Due to small sample sizes, only four model categories were estimated with ABC-C UI score categories:
- Likelihood of ≥0 (versus <0) specialist visits in the past year
- Likelihood of ≥0 (versus <0) medical tests or procedures currently used
- Likelihood of high (versus low) financial burden
- Likelihood that a family member did (versus did not) quit working as a result of FXS-related caregiving

Results indicated that compared with those in the very high ABC-C UI score group, those in the low ABC-C UI score group were significantly more likely to have ≥3 (versus ≤2) specialist visits in the past year (odds ratio = 10.2; P = 0.0044) and have high (versus low) financial burden related to FXS caregiving (odds ratio = 9.5; P = 0.0146)

Limitations
- Respondents in this survey may not be representative of the entire universe of FXS caregivers in the US, and results may not be generalizable to FXS caregiving situations in countries with different health care systems.
- Use of a survey format allows for data to be collected from a large sample but also necessitates a limited amount of response options and follow-up questions that can be included to further describe caregiver burden

Conclusions
- The ABC-C UI appears to function well as an indicator of health-related economic burden and quality of life in individuals with FXS and their caregivers
- Estimates of higher ABC-C UI values appear to be associated with more favorable outcomes at the individual patient and caregiver level
- Health care providers, payers, and other key decision makers may find these results useful in the overall assessment of the value of new treatments for patients with FXS

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References

Table 1. Participant Characteristics, Overall and by ABC-C UI Score Category

Table 2. Burden of Illness Summary, Overall and by ABC-C UI Score Category