Brief Report: Assessment of Children’s Gastrointestinal Symptoms for Clinical Trials

**Lynn S. Walker,** PhD, and **Susan C. Sorrells,** PharmD

*Vanderbilt University School of Medicine and GlaxoSmithKline, Inc.*

**Objective:** To conduct a pilot study evaluating a procedure for assessment of daily symptoms and functioning in pediatric patients.

**Method:** Participants included 11 parent-child dyads referred to a tertiary care center for evaluation of constipation and abdominal pain. Each family was provided a hand-held computer and modem. For 7 consecutive days, parents and children (ages 6–10 years) responded as a team to questions regarding the level of children’s gastrointestinal symptoms and the extent to which symptoms interfered with the day’s activities. Parents responded to a telephone interview evaluating the procedure.

**Results:** Parents reported that children understood most questions and that responses entered into the computer were accurate. Parents and children were enthusiastic about the data collection method. Some technical problems arose in use of the computers.

**Conclusions:** Within the limitations of a small sample, this data collection procedure appears to have promise for evaluating pediatric symptom outcomes.

**Key words:** clinical trials; irritable bowel syndrome; electronic diary; quality of life; children.

In 1998, the U.S. Food and Drug Administration published the “Pediatric Rule” requiring the pharmaceutical industry to include safety and efficacy data in the pediatric population for new drug applications (Food and Drug Administration, 1998). As a result, clinical outcome trials evaluating drug efficacy and safety in pediatric populations are likely to increase. These trials in adults often entail symptom assessment on a daily basis with paper and pencil or an electronic touch-tone telephone system (e.g., Jones et al., 1999). Developmentally appropriate tools will be needed for upcoming clinical trials in children and adolescents.

This pilot and feasibility study examined a data collection tool in a pediatric population with symptoms of irritable bowel syndrome (IBS). IBS, a functional gastrointestinal disorder, was a particularly appropriate focus for the study because the absence of biological indicators for IBS means that measures of treatment outcome must rely exclusively on patients’ reports of their symptoms (Drossman, 1994). Recent studies have demonstrated that symptoms associated with IBS affect a significant number of children and adolescents (Hyams et al., 1996; Walker, Guite, Duke, Barnard, & Greene, 1998). New drugs for the treatment of IBS have been evaluated in adults (e.g., Camilleri et al., 1999) but have not yet been tested in children. Neither the assessment questions nor the data collection tools in the adult outcome studies are appropriate for children.
In collecting data from children, one must address limitations in children's memory, vocabulary, attention span, and ability to understand complex sentence structure (Blair, 2000). Zeltzer and colleagues demonstrated that children as young as 6 years of age can use a rating scale to quantify their somatic sensations (Zeltzer et al., 1988). However, assessment questions and response options for children must be simple and the reference period for recall must be short. In addition, the format of protocols and the data collection tools must appeal to children.

Because of these challenges in collecting data directly from children, their symptoms often are assessed by proxy from parents. This approach has drawbacks in that parents may not be aware of children's symptoms, particularly those not manifest in observable behavior or that occur during school hours (Garber, Van Slyke, & Walker, 1998). Thus, parent reports of children's symptoms may not accurately reflect drug efficacy.

This study aimed to address these data collection issues by generating developmentally appropriate questions for assessing children's symptoms of IBS and by using a hand-held computer and parent-child team approach, rather than individual parent or child reports, to assess children's symptoms. Use of a parent-child team approach was based on the premise that pediatric symptom reports obtained by health care professionals often represent a collaborative effort by parent and child to provide the most accurate data possible. Although physicians may direct many of their questions to parents, during the course of the clinical interview it is common for the parent or physician to consult with the child, particularly regarding subjective, nonobservable symptoms. This study formalized clinical practice with a parent-child team approach to data collection. Specifically, parents and children were instructed to consult with each other as they responded to assessments of children's symptoms and functioning on 7 consecutive days.

Data collection occurred by means of a hand-held computer similar to that of popular electronic games. In addition to enhancing the study's appeal to children, electronic diaries have advantages over paper diaries, including improved data quality and elimination of participants' ability to “backfill” diaries to appear compliant (Shiffman, Hufford, & Paty, 2001). Electronic data collection devices have been used in studies of adolescent behavior (e.g., Whalen, Jamner, Henker, & Delfino, 2001), but are less commonly used with young children. We describe results of a parent interview evaluating this procedure for assessment of children's gastrointestinal symptoms and daily functioning.

Method

Sample and Procedure

Participants were 11 children between 6 and 10 years of age (median = 8 years) who were referred to a university medical center for evaluation of constipation and abdominal pain, two of the cardinal symptoms associated with IBS. Children with other chronic medical conditions (e.g., diabetes) were excluded. All children were Caucasian. Ten mothers and one father participated. Of the 12 families invited to participate, 1 family declined due to time constraints. Vanderbilt University's Institutional Review Board approved the study. Parents provided written consent and children gave written consent (ages 8 and older) or verbal assent (ages 6 and 7).

Following the medical evaluation, children and their parents were trained in use of the hand-held computer (the Personal Data Assistant, PDA). They were instructed to operate as a “team” in answering the questions; that is, parents and children were to consult with each other in answering each question on the computer screen. Families were given a PDA to take home. In the evening on the next 7 days, they responded to questions regarding the level of the child’s symptoms and the extent to which those symptoms interfered with the child’s activities that day. The PDA was placed in a receiver overnight to allow transmission of data to a central data bank. Following the final PDA assessment, parents responded by telephone to questions evaluating the protocol.

Measures

Gastrointestinal Symptoms. Nine questions were generated to assess the indicators of treatment efficacy used in studies of adults with IBS (Camilleri et al., 1999; Mangel et al., 1998). The principal measure of efficacy in the adult studies was the question, “Have you had adequate relief of your irritable bowel syndrome pain and discomfort today?” This question was reworded as two items assessing the key components of IBS: (1) abdominal discomfort
(“Did your tummy feel OK today?”) and, (2) bowel dysfunction (“Was your pooping OK today?”). Additional questions assessed secondary indicators of treatment efficacy. Wording of these questions was derived from the child-report form of the Questionnaire on Gastrointestinal Symptoms (Walker, Caplan-Dover, & Rasquin-Weber, 2000). Questions assessed the extent of discomfort (“How much did your tummy hurt or feel uncomfortable today?”); frequency of bowel movements (“How many times did you poop today?”); stool consistency (“What was your poop like today?”); bowel urgency (“Did you feel like you had to rush to the bathroom to poop today?”); abdominal bloating (“Did your tummy feel swollen or puffy today?”); and incomplete evacuation (“After you finished pooping today, did it feel like there was still more poop that didn’t come out?”). Finally, impact on daily functioning was assessed with the question, “Did you miss any school or playtime today because of problems with your tummy or pooping?”

Evaluation of Research Protocol. Parents were interviewed by telephone regarding children’s understanding of questions, ease of computer use, accuracy of responses, level of child participation, and satisfaction.

Equipment

Equipment consisted of a PDA (Compaq Aero 1500 Series) with a receiver and modem that could be plugged into a telephone jack. The computer emitted a beep at 8 p.m. if the assessment had not been completed by that time. Each day, the computer accepted responses to the questions until midnight (if no responses were recorded by that time, data for that day were considered missing). Data were transmitted to the central data bank during the night. Families were given a toll-free telephone number for technical support in case of difficulty using the PDA.

Results

Children’s Comprehension of Questions Assessing Gastrointestinal Symptoms. Based on parent report, six of the nine questions were understood by all children. The most difficulty was reported for the item assessing abdominal bloating. Parents of 4 of the 11 children reported that their children did not seem to understand this question. Parents of two children indicated that their children did not understand the item assessing stool consistency (both parents reported that their children said they had not touched their stool and therefore did not know what it was like). Finally, one parent was unsure whether her child understood the item assessing incomplete evacuation.

Ease of Computer Use. Parents reported that the daily assessment took little time (M = 3.63 minutes, range = 2–10 minutes). It interfered “not at all” (n = 10) or “a little” (n = 1) with family activities. Finally, using the computer was “not at all difficult” (n = 10) or “a little difficult” (n = 1).

Accuracy of Responses. Parents believed that the responses entered into the PDA were “very accurate” (n = 8) or “accurate” (n = 3).

Child and Parent Team. Ten of the eleven parents reported that both they and their child were present for all of the daily assessments (one parent reported that her child completed the assessment himself one evening when she was not home). Regarding any difficulty in getting children to take the time to complete the assessment, ten parents reported no difficulty and one parent reported that it was “a little difficult.”

Parents reported that their children needed little assistance in answering the questions. Specifically, one parent reported that she helped her child with symptom recall “several times,” and the remaining parents reported that they helped their children “once or twice.”

Satisfaction with the Protocol. All parents reported that both they and their children liked answering the computer questions.

Willingness to Participate in Future Outcome Studies. Parents indicated that they and their children would be willing to participate in a similar study in the future. Regarding the duration of participation, 9 parents believed that their children would participate for 10 weeks or longer, and all 11 parents believed that their children would participate for at least 3 weeks.

Qualitative Data from Parent Interviews. Parents were encouraged to comment about their experience with the study. Examples of these comments, which were consistently positive, include the following: “He loved doing it.” “He reminded me every night and could hardly wait to do it.” “It was something that was hers and was important to her.” “It made her feel important.” “It was easy even for someone who didn’t have knowledge of computers.” In addition, several parents indicated that they appreciated the daily assessment because it helped
them learn about their children’s symptoms, for example, “I got to find out things that she might not ordinarily say to me.”

**Compliance.** Each participating family completed all items on each of the 7 days of their participation. Thus, there were no missing data. In three cases, the 7 days were not consecutive; this was due to technical problems with the computer (see below).

**Technical Problems.** Three families had trouble logging onto the computer and called technical support for instructions on how to reset the computer. One modem malfunctioned and did not transmit data; these data were stored in the computer and retrieved when the computer was returned. One computer did not function properly, and the family was sent another computer by mail. Following completion of the study, families returned the PDA in a prepaid mailer. One of the computers was never received, and it was not possible to trace it (the data, however, had been transmitted by modem and were not lost). The batteries in two computers ran down in storage and the program had to be re-entered prior to giving the computers to the families.

**Discussion**

The procedure described in this study may be useful in pediatric clinical trials that require subjective reports of symptoms and daily functioning for the evaluation of treatment efficacy. Using symptoms of IBS as an example, the study showed that it is possible to reword treatment efficacy questions from adult studies so that they can be understood by children as young as 6 years of age. The single possible exception in this study was the symptom of bloating, which was not understood by four children. It should be noted that this symptom might be so rare among children that lack of familiarity would hamper their comprehension even if the question assessing bloating were reworded. Reliability and validity of the symptom measures should be investigated with a larger sample.

Use of the computer appealed to children and may have motivated their compliance with daily reporting for the week. It is likely that data quality was improved by direct entry into the computer rather than transfer from paper diaries. The problem of patients recording daily symptoms retrospectively, often encountered in paper diaries, was not an issue because the computer accepted each day’s data only until midnight of that day. In addition, it is possible that children gave more information to the computer than would have been the case with a personal interview or paper diary that might have been perceived as less confidential (cf. Holt, Guram, Smith, & Skinner, 1992). There were no missing data. A drawback of the computer was that several families encountered problems that required telephone contact with technical support staff. Availability of such support is critical to the success of studies using electronic diaries.

Finally, the parent-child team approach was well accepted by parents and children and yielded data participating parents regarded as highly accurate. Spontaneous comments by the parents were uniformly positive and enthusiastic. Both parents and children enjoyed the opportunity for the brief structured time when they reviewed the day together and assessed the children’s symptoms. Parents reported that they and their children would be willing to participate again in a similar study of considerably longer duration. This is important, as clinical trials often require several weeks of data collection.

This pilot and feasibility study was limited by a small sample size. Nonetheless, results suggest that this data collection approach may hold promise for other pediatric treatment studies in which children’s symptoms are an outcome measure. Additional study with a larger sample is needed to compare this approach to others with respect to cost, data quality, and participant satisfaction and retention.

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