

Development and content validity of an observer-reported outcome (ObsRO) diary for use among caregivers of children with eosinophilic esophagitis



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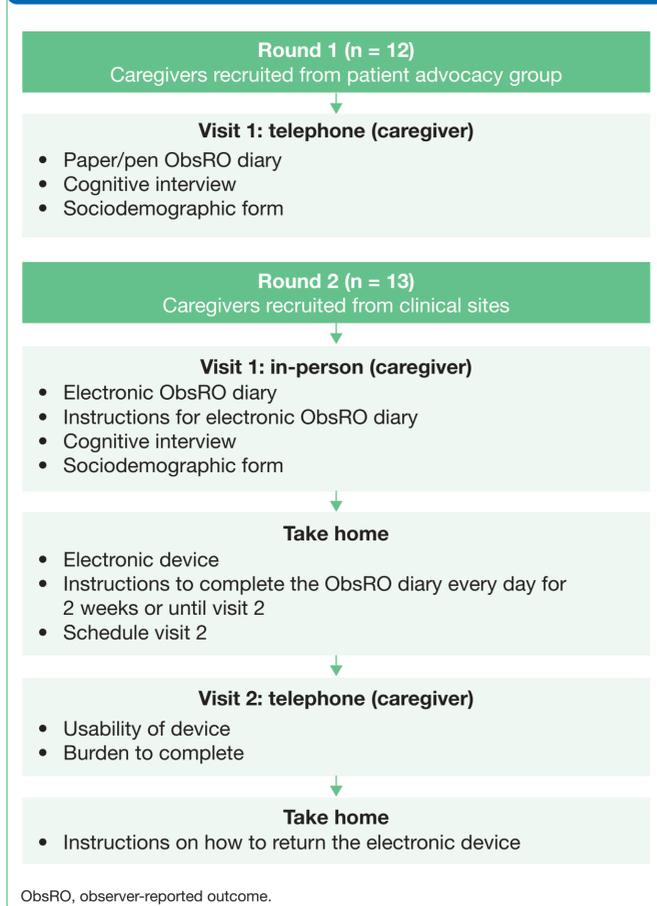
INTRODUCTION

- Eosinophilic esophagitis (EoE) is a chronic, immune-mediated disease characterized by symptoms of esophageal dysfunction and eosinophilic inflammation.¹
- EoE causes reflux-like symptoms and feeding problems in children, often differing from adult symptom presentation.¹
- Symptoms are best reported by the patients themselves. However, for young children who are unable to self-report, the use of an observer-reported outcome (ObsRO) diary by caregivers is encouraged to record observable events or behaviors.²
- This research investigated the content validity of an ObsRO diary being developed for use among caregivers of children (aged 2–10 years) with EoE. The usability of the ObsRO diary on an electronic handheld device was also assessed.

METHODS

- This was a qualitative, observational study involving two rounds of interviews, each with a separate cohort of caregivers (Figure 1).
 - Round one consisted of a cognitive interview by telephone, which aimed to:
 - elicit concepts related to EoE (observable behaviors and verbalizations related to signs and symptoms of EoE)

Figure 1. Study design



- assess the completeness, relevance and comprehensibility of the ObsRO diary.
- Round 2 consisted of two interviews with a second cohort, which assessed:
 - the content validity of the ObsRO diary (visit 1, in-person)
 - the usability of the electronic platform (visit 2, by telephone).
- Informed consent was obtained from participants during each round.
- Caregivers were recruited from the patient advocacy group Campaign Urging Research for Eosinophilic Disease (CURED) and two clinical sites in the USA, for round 1 and round 2, respectively.
- All caregivers were recruited and screened via telephone using a standardized script.
- The ObsRO diary investigated in this study was developed according to guidance provided by the US Food and Drug Administration ("Patient-reported outcome measures: use in medical product development to support labeling claims").³

RESULTS

Participant demographics and characteristics

- Overall, 25 caregivers were enrolled (round 1, n = 12; round 2, n = 13).
 - All caregivers were mothers of children with EoE; their mean (standard deviation [± SD]) age was 36.8 (± 5.5) years.

Table 1. Demographics and characteristics of children with eosinophilic esophagitis based on caregiver report

Demographics/characteristics	Overall (N = 25)
Age, years	
Mean (± standard deviation)	7.1 (± 2.4)
Median (range)	7.0 (3.0–10.0)
Male, n (%)	19.0 (76.0)
Ethnicity, n (%)	
Hispanic	3 (12.0)
Not Hispanic or Latino	22 (88.0)
Race, n (%)	
White	20 (80.0)
Black or African-American	1 (4.0)
Other	4 (16.0)
Age at diagnosis, years	
Mean (± standard deviation)	4.5 (± 2.6)
Median (range)	4.0 (0.0–10.0)
Education level, n (%)	
No schooling outside of the home	2 (8.0)
Daycare or preschool program outside of the home	3 (12.0)
Primary/elementary school: kindergarten	3 (12.0)
Primary/elementary school: 1st–6th grades	14 (56.0)
Primary/elementary school: grade unknown	2 (8.0)
Other	1 (4.0)
Difficulty with swallowing, n (%)	
Yes	17 (68.0)
No	8 (32.0)

Table 2. Concepts related to eosinophilic esophagitis according to caregivers' reports of signs, symptoms and observable behaviors

Concept, n (%)	Total reports (N = 25)	Reports according to age of child			
		2–4 years (N = 5)	5–6 years (N = 5)	7–8 years (N = 7)	9–10 years (N = 8)
Stomach pain	21 (84.0)	5 (100.0)	4 (80.0)	6 (85.7)	6 (75.0)
Avoiding or refusing food	15 (60.0)	4 (80.0)	3 (60.0)	4 (57.1)	4 (50.0)
Difficulty with swallowing	13 (52.0)	2 (40.0)	2 (40.0)	4 (57.1)	5 (62.5)
Heartburn/reflux	12 (48.0)	2 (40.0)	1 (20.0)	5 (71.4)	4 (50.0)
Vomiting	9 (36.0)	1 (20.0)	3 (60.0)	1 (14.3)	4 (50.0)
Nausea	8 (32.0)	1 (20.0)	1 (20.0)	3 (42.9)	3 (37.5)
Being full too early	8 (32.0)	3 (60.0)	3 (60.0)	2 (28.6)	0 (0.0)
Chest pain	7 (28.0)	1 (20.0)	1 (20.0)	2 (28.6)	3 (37.5)
Diarrhea	6 (24.0)	0 (0.0)	2 (40.0)	0 (0.0)	4 (50.0)
Regurgitation	5 (20.0)	0 (0.0)	1 (20.0)	4 (57.1)	0 (0.0)
Burping or belching	5 (20.0)	1 (20.0)	0 (0.0)	3 (42.9)	1 (12.5)
Failure to thrive	4 (16.0)	1 (20.0)	2 (40.0)	0 (0.0)	1 (12.5)
Coughing	4 (16.0)	1 (20.0)	0 (0.0)	0 (0.0)	3 (37.5)
Sleep disturbance	3 (12.0)	2 (40.0)	0 (0.0)	1 (14.3)	0 (0.0)
Choking	3 (12.0)	0 (0.0)	2 (40.0)	0 (0.0)	1 (12.5)
Throat tightness	2 (8.0)	0 (0.0)	0 (0.0)	1 (14.3)	1 (12.5)
Constipation	2 (8.0)	0 (0.0)	0 (0.0)	1 (14.3)	1 (12.5)
Tall/skinny/not growing	1 (4.0)	1 (20.0)	0 (0.0)	0 (0.0)	0 (0.0)
Drinks a lot of water	1 (4.0)	1 (20.0)	0 (0.0)	0 (0.0)	0 (0.0)
Rash	1 (4.0)	0 (0.0)	1 (20.0)	0 (0.0)	0 (0.0)
Fatigue	1 (4.0)	0 (0.0)	0 (0.0)	0 (0.0)	1 (12.5)
Itching	1 (4.0)	0 (0.0)	0 (0.0)	1 (14.3)	0 (0.0)

- The demographics and disease characteristics of the children with EoE are shown in Table 1.
 - The mean (± SD) age of the patients was 7.1 (± 2.4) years.
 - Most caregivers (n = 17, 68%) indicated in the sociodemographic form that their children had previously experienced difficulty with swallowing.

Content validity and usability of the ObsRO diary

- In round 1, concept saturation, the point at which no new concepts emerge, was achieved during the 12th interview, with 22 unique concepts identified (Table 2).
 - The most commonly reported concepts were stomach pain (n = 21, 84%), avoiding or refusing food (n = 15, 60%), difficulty with swallowing (n = 13, 52%) and heartburn/reflux (n = 12, 48%).
- Participants were asked "If your child were to begin a new treatment for EoE, how would you know that the treatment was working?" The majority of participants replied that indicators of a treatment response would be:
 - reduction in symptom severity, frequency and duration
 - signs that their children were eating more
 - signs that their children's overall health seemed to be improving.
- All 25 respondents confirmed that the questions in the ObsRO diary were relevant for their children with EoE and were easy to understand, using comments such as "good," "easy to follow" and "it gives a good view on a lot of the major symptoms of EoE that children experience."
- In round 2, the majority of caregivers (11/12, 92%) reported that the electronic format of the ObsRO diary was acceptable and easy to complete.
 - Most participants (10/12, 83%) were willing to complete the ObsRO diary on a daily basis, and most (9/12, 75%) did not have trouble remembering to complete it.

CONCLUSIONS

- In the absence of self-reporting, the use of an ObsRO instrument is essential to ensure that the patient experience is included in treatment assessments.
- This study supports the content validity and electronic usability of this ObsRO diary being developed for caregivers of children with EoE.
- Following interviews with caregivers of children with EoE, this study has identified concepts that are consistent with the heterogeneity of the signs and symptoms previously reported in children with EoE.¹
- This ObsRO diary was developed as per guidance from the US Food and Drug Administration.³ Further development of the instrument is required to determine its psychometric properties and performance (reliability, validity and ability to detect meaningful change).³

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DISCLOSURES

BG, NKD and JW are employees of Shire, a member of the Takeda group of companies and stockholders of Takeda. RP, AB and AB are employees of Evidera.

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