

Patient-centered measures for achalasia

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Abstract

Background Various instruments may be used to measure health-related quality of life in patients with achalasia.

Methods We administered four patient-centered measures used for evaluation of achalasia severity [an achalasia severity questionnaire we developed previously, an achalasia symptom checklist, the Gastrointestinal Quality-of-Life Index (GIQLI), and the Medical Outcomes Study 36-item Short-Form survey (SF-36)] to 25 subjects enrolled in a randomized controlled trial comparing pneumatic dilatation and laparoscopic Heller myotomy. We estimated correlations between the different measures.

Results Twenty-five patients (13 male, 12 female) were studied; 12 were treated by pneumatic dilatation and 13 by laparoscopic myotomy. The average age of patients was 48.5 [range 25–69, standard deviation (SD) 13.7] years. Baseline scores demonstrated a substantial burden of impairment. The mean (SD) score on the achalasia severity measure [ranges from 0 (best) to 100 (worst)] was 62.3 (13.4). The mean (SD) symptom checklist score [ranges from 0 (best) to 36 (worst)] was 23.2 (6.6). The mean (SD) GIQLI [ranges from 0 (worst) to 144 (best)] was 77.04 (19.4). The SF-36 mean (SD) for the physical component score (PCS) was 45.29 (9.21) and the mean for the mental component score (MCS) was 37.61 (14.97). The achalasia severity measure correlated highly with the GIQLI ($r = -0.57$, $p = 0.01$), and the symptom checklist ($r = 0.65$, $p = 0.004$). The achalasia severity measure correlated well with the SF-36 PCS ($r = -0.42$, $p = 0.039$), but not with the MCS ($r = -0.14$, $p = 0.501$).

Conclusion Subjects recruited to a randomized controlled trial of achalasia treatment demonstrated impairment in both generic quality-of-life and disease-specific measures. Scores on achalasia-specific measures correlated well with each other, but less well with measures of generic quality-of-life and mental health scales. Because of the multidimensional nature of achalasia, disease-specific measures should be combined with generic health measures for the best assessment of patient outcome.

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Achalasia is a rare esophageal disease characterized by the failure of the lower esophageal sphincter to relax upon swallowing and the absence of peristalsis in the esophageal

body. Pneumatic dilatation and laparoscopic Heller myotomy are the two most common, long-term treatments for this disease. Since achalasia is not curable; treatment is intended to relieve symptoms and improve well being. A variety of methods to measure treatment response for achalasia have been used.

Health-related quality-of-life measures (HRQoL) are often used in assessing the outcome of medical treatment. Low HRQoL is often correlated with symptom severity. HRQoL, in gastrointestinal disease in general and achalasia in particular, is dependent on the patient's interpretation of symptom relief, and the impact of that relief on their sense of well being [1].

In the area of gastrointestinal diseases, numerous measures exist to study irritable bowel syndrome and gastroesophageal reflux disease [1, 2], but very few measures exist to assess HRQoL for achalasia. We developed and validated the ten-item achalasia severity questionnaire (ASQ) to fill this void [3]. Because the ASQ measures social dimensions of health, as well as health perceptions, it may be considered a disease-specific measure of quality of life. This paper: (1) examines four instruments used to assess patients with achalasia enrolled in a randomized controlled trial comparing pneumatic dilatation and laparoscopic Heller myotomy with partial fundoplication; and (2) analyzes how well disease-specific measures correlate with generic measures of HRQoL.

Methods

Patients

As part of a randomized controlled trial, patients with achalasia were recruited from the motility laboratory and from the offices of local gastroenterologists and surgeons. All patients had a clinical diagnosis of achalasia, confirmed with manometry, endoscopy, and a timed barium esophagram. Prior to treatment, all patients completed a set of baseline questionnaires. The clinical trial is still ongoing, and we do not report any outcomes of the clinical trial in this paper. The study protocol was approved by the University Health Network research ethics board and all patients gave informed consent before participating in the trial.

Instruments

ASQ: This is a disease-specific instrument with ten items that sample the concepts of food tolerance, dysphagia-related behavior modifications, pain, heartburn, distress, lifestyle limitation, and satisfaction [3]. The measure

results in scores on an interval scale of 1 to 100, with 100 representing the worst possible score (highest level of severity).

Symptom checklist: This is a six-item checklist rating the frequency and severity of symptoms on a scale from zero to three and was developed to address the range of typical clinical symptoms experienced by patients with achalasia. Patients were asked to rate both the frequency and severity of dysphagia, odynophagia, heartburn, food regurgitation, acid regurgitation, and chest pain. Scores could range from 0 (best) to 36 (worst).

GIQLI: This is an instrument specifically for patients with gastrointestinal disease, with 36 items rated on a five-point Likert scale from zero to four [4]. Responses are then added together to give a numerical score with a range from 0 (worst) to 144 (best). A normal score for the GIQLI is 125 [4].

Medical Outcomes Study SF-36: This is a generic instrument designed to measure generic HRQoL. The SF-36 has 36 questions covering eight domains of physical functioning including physical and mental role limitations, bodily pain, mental health, social functioning, vitality, and general health perceptions [5]. Scores range from 0 to 100, with a higher score representing better health status. In this study, we report two component scores of the SF-36: (1) the physical component score (PCS); and (2) the mental component score (MCS). The PCS and MCS of the SF-36 have a mean normative value of 50 and a standard deviation of 10 in the general population, therefore scores above 50 are better than the general population average, while scores below 50 are worse [6].

Data analysis

The data were analyzed using SPSS software (SPSS Inc, Chicago IL). Descriptive statistics were tabulated for patient variables, and the mean scores and standard deviations were calculated for each of the instruments. Pearson's correlation coefficient was calculated to estimate the relationship between the measures. A *p*-value of less than 0.05 was considered statistically significant.

Results

Patients

At the time this study was done, there were 25 patients with achalasia enrolled in the clinical trial (23 primary, 2 recurrent), with a mean age of 48.5 (range 25–69, Table 1). Thirteen (52%) of the patients were men. The two recurrent patients had both had pneumatic dilatations at least one

Table 1 Demographics

Variable	Value
Gender <i>N</i> , (%)	
Male	13 (52)
Female	12 (48)
Age (years)	
Mean (standard deviation)	48.5 (13.71)
Range	25–69

year previously. No patients had been previously treated with botulinum toxin. Thirteen (52%) of the patients were assigned to laparoscopic Heller myotomy.

HRQoL and disease-specific measures

The baseline mean scores for all measures are presented in Table 2. Baseline scores demonstrated a substantial burden of impairment. The mean score for patients on the ASQ was 62.32 (SD 13.43). Patients also completed a symptom severity checklist with a mean score of 23.24 (SD 6.57). The mean (SD) GIQLI score was 77.04 (19.44). The SF-36 PCS mean (SD) score was 45.29 (9.21), and the MCS mean (SD) score was 37.61 (14.97).

Correlations

Table 3 shows the correlations between the ASQ and the other measures. The ASQ correlated highly with the symptom checklist (0.65, $p < 0.01$) and the GIQLI (−0.57,

Table 2 Scores on patient-centered measures

Instrument	Mean	Standard deviation	Possible range of instrument	
			Best	Worst
ASQ	62.32	13.43	0	100
Symptom checklist	23.24	6.57	0	36
GIQLI	77.04	19.44	144	0
SF-36				
PCS	45.29	9.21	NA*	NA
MCS	37.61	14.97	NA	NA

ASQ, Achalasia Severity Questionnaire

GIQLI, Gastrointestinal Quality-of-Life Index

SF-36, Medical Outcomes Study 36-item Short-Form Health Survey

NA, not applicable

*Physical and mental component summary scores use norm-based scoring, with a mean of 50 and standard deviation of 10, with higher values indicating better quality of life

Table 3 Correlations

Variables	ASQ	Symptom checklist	GIQLI	SF-36 PCS	SF-36 MCS
ASQ	—				
Symptom checklist	0.64**	—			
GIQLI	−0.57**	−0.51**	—		
SF-36 PCS	−0.424*	−0.496*	−0.514*	—	
SF-36 MCS	−0.144	−0.282	0.635**	0.68	—

ASQ, Achalasia Severity Questionnaire

GIQLI, Gastrointestinal Quality-of-Life Index

SF-36, Medical Outcomes Study 36-item Short-Form Health Survey

* $p < 0.05$, ** $p < 0.01$

$p < 0.01$), but not with the SF-36 MCS (−0.144, $p = 0.501$). Scores on the SF-36 MCS correlated highly with GIQLI (0.635, $p < 0.01$).

Discussion

Dysphagia and other achalasia symptoms are difficult to quantify [7], and studies vary in their definition of a poor treatment outcome [8]. Previous studies have tended to focus only on specific symptoms, while ignoring dimensions of HRQoL, such as health perceptions, and social and physical limitations [9, 8]. Depending on the criteria for determining the success or failure of treatment, patients may be satisfied with the procedure, but still be classified as treatment failures or as having a poor outcome based on symptoms [8] or follow-up testing [10]. Analysis of only one dimension will not capture the range of effects the disease and treatment have on overall well being. The ASQ includes dimensions of health status, such as symptoms, as well as dimensions of HRQoL, such as health perceptions, social and lifestyle limitations, and coping strategies [3]. When we combine this disease-specific measure with other generic measures of health related quality of life, we get a better understanding of the patient's perspective of their disease.

The measures used in this study show that patients with newly diagnosed achalasia have poor function and impaired HRQoL. With respect to the GIQLI, our patients scored well below the mean score for normal individuals (77 versus 125) [4], and worse than patients with symptomatic gallstone disease (87) [4] or esophageal cancer (89) [4]. Our patients also had poor overall HRQoL functioning, as measured by the SF-36. In other surveys, persons with serious medical problems scored 49.13 for general health perception [5]. Patients with achalasia score poorly in the areas of physical role limitations because of the inability to eat and maintain good nutrition [11]. Achalasia patients

also scored poorly in the mental health domain, demonstrating significant trouble coping with their illness.

The main finding of this study was the observation that the ASQ correlated well with symptom scores, but was less correlated with generic measures of HRQoL, particularly in the area of mental health. This is not unexpected, since generic measures capture many dimensions of health, some of which may not be markedly impaired by one particular disease process. However, it is important to recognize the multidimensional nature of impairment in achalasia, and also the consequences of different types of therapeutic interventions, which may have broad and unpredictable effects on quality of life. Therefore, it is advisable to use both disease-specific measures as well as generic measures of HRQoL in the evaluation of treatment for achalasia.

The main limitation of our study is our small sample size, and the fact that our sample may not be representative of all achalasia patients. We have recruited only 25 patients thus far and it is possible we may not be capturing those patients with less severe symptoms or patients in the early stages of the disease.

Achalasia is a chronic esophageal disease that affects the quality of life of patients on many levels. The multidimensional nature of the disease requires a multidimensional strategy for measuring the success or failure of medical treatment. There are a number of resources available for studies of patients with achalasia. Because of the multidimensional nature of impairment in achalasia, we recommend that disease-specific measures should be combined with generic HRQoL measures to get the most comprehensive assessment of patient outcome.

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References

1. Revicki DA, Wood M, Wiklund I, Crawley J (1998) Reliability and validity of the gastrointestinal symptom rating scale in patients with gastroesophageal reflux disease. *Qual Life Res* 7:75–83
2. Damiano A, Handley K, Adler E, Siddique R, Bhattacharyya A (2002) Measuring symptom distress and health-related quality of life in clinical trials of gastroesophageal reflux disease. *Dig Dis Sci* 47:1530–1537
3. Urbach DR, Tomlinson GA, Harnish JL, Martino R, Diamant NE (2005) A measure of disease-specific health-related quality of life for achalasia. *Am J Gastroenterol* 100:1–9
4. Eypasch E, Williams JJ, Wood-Dauphinee S, Ure BM, Schumling C, Neugebauer E, Troidl H (1995) Gastrointestinal quality of life index: Development, validation and application of a new instrument. *Br J Surg* 82:216–222
5. McHorney CA, Ware JE, Raczek AE (1993) The MOS 36-item short-form health survey (SF-36): II psychometric and clinical tests of validity in measuring physical and mental health constructs. *Med Care* 31:247–263
6. Ware JE, Kosinski M (2001) SF-36 Physical and mental health summary scales: A manual for users of version 1 (2nd Edition). QualityMetric Inc., Lincoln, RI
7. Dakkak M, Bennett JR (1992) New dysphagia score with objective validation. *J Clin Gastroenterol* 14:99–100
8. Wills VL, Hunt DR (2001) Functional outcome after Heller myotomy and fundoplication for achalasia. *J Gastrointest Surg* 5:408–413
9. West RL, Hirsch DP, Bartelsman JFWM, De Borst J, Ferwerda G, Tytgat GNJ, Boeckxstaens GE (2002) Long-term results of pneumatic dilatation in achalasia followed for more than 5 years. *Am J Gastroenterol* 97:1346–1351
10. Blam ME, Delfyett W, Levine MS, Metz DC, Katzka DA (2002) Achalasia: A disease of varied and subtle symptoms that do not correlate with radiographic findings. *Am J Gastroenterol* 97:1916–1923
11. Ben-Meir A, Urbach DR, Khajanchee YS, Hansen PD, Swanson LL (2001) Quality of life before and after laparoscopic Heller myotomy for achalasia. *Am J Surg* 181:471–474