Patient-Reported Outcomes in Esophageal Diseases

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In my introductory comments to the practice management section last year, I wrote about cultivating competencies for value-based care. One of the key competencies was patient centeredness. Patient-reported outcomes (PROs) and patient experience measures specifically were highlighted as examples of meaningful tools for achieving patient centeredness. Starting with this month's contribution by Drs Reed and Dellon on PROs in esophageal disease, we begin a series of articles focused on this important construct. We will follow this article with reports focused on PRO for patients with irritable bowel syndrome, inflammatory bowel disease, and chronic liver disease. These reports will not only review the importance of PROs, but also highlight the most practical approaches to measuring disease-specific PROs in clinical practice all with the goal of improving the care of our patients.

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Patients seek medical care for symptoms affecting their quality of life,[1] and this is particularly true of digestive diseases, in which many common conditions are symptom-predominant. However, clinician and patient perception of symptoms often conflict,[2] and formalized measurement tools may have a role for optimizing symptom assessment. Patient-reported outcomes (PROs) directly capture patients' health status from their own perspectives and can bridge the divide between patient and provider interpretation. The US Food and Drug Administration (FDA) defines PROs as "any report of the status of a patient's health condition that comes directly from the patient without interpretation of the patient's response by a clinician or anyone else."[3]

For the clinical assessment of esophageal diseases, existing physiologic and structural testing modalities cannot ascertain patient disease perception or measure the impact of symptoms on health care–associated quality of life. In contrast, by capturing patient-centric data, PROs can provide insight into the psychosocial aspects of patient disease perceptions; capture health-related quality of life (HRQL); improve provider understanding; highlight discordance between physiologic, symptom, and HRQL measures; and formalize follow-up evaluation of treatment response.[1,4] Following up symptoms such as dysphagia or heartburn over time in a structured way allows clinically obtained data to be used in pragmatic or comparative effectiveness studies. PROs are now an integral part of the FDA's drug approval process.

In this article, we review the available PROs capturing esophageal symptoms with a focus on dysphagia and heartburn measures that were developed with rigorous methodology; it is beyond the scope of this article to perform a thorough review of all upper gastrointestinal (GI) PROs or quality-of-life PROs. We then discuss how esophageal PROs may be incorporated into clinical practice now, as well as opportunities for PRO use in the future.

Esophageal Symptom-Specific Patient-Reported Outcomes

The literature pertinent to upper GI and esophageal-specific PROs is heterogeneous, and the development of PROs has been variable in rigor. Two recent systematic reviews identified PROs pertinent to dysphagia and heartburn (Table 1) and both emphasized rigorous measures developed in accordance with FDA guidance.[3]

Patel et al[5] identified 34 dysphagia-specific PRO measures, of which 10 were rigorously developed (Table 1). These measures encompassed multiple conditions including esophageal cancer (Functional Assessment of Cancer Therapy Esophageal Cancer Subscale, European Organization for Research and Treatment of Cancer Quality-of-Life with esophageal Cancer 25 items, European Organization for Research and Treatment of Cancer Quality-of-Life with esophageal cancer 18 items, upper aerodigestive neoplasm-attributable

Abbreviations used in this paper: **FDA, Food and Drug Administration; GERD, gastroesophageal reflux disease; GI, gastrointestinal; HRQL, health-related quality of life; PRO, patient-reported outcome; PROMIS, Patient-Reported Outcomes Measurement Information System.**

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Table 1. Overview of Esophageal-PROs for Measuring Dysphagia or Heartburn

Condition or symptom and instrument	Target population	Longitudinal validity	Plan for scoring measure and missing data	Reference
Esophageal cancer				
FACT-E[a]	Cohort A: adults with resectable squamous or adenocarcinoma of the esophagus or GEJ Cohort B: esophageal cancer patients with planned neoadjuvant chemoradiation before surgery	Yes	Yes/no	Cancer 2006;107:854–863[13]
EORTC-QLQ-OG25	Esophageal or gastric cancer including tumors of the GEJ	No	Yes/no	Eur J Cancer 2007;43:2066–2073[14]
EORTC-QLQ-OES18	Newly diagnosed squamous cell or esophageal adenocarcinoma	No	Yes/no	Eur J Cancer 2003;39:1384–1394[15]
Cancer-attributed OP dysphagia				
MDADI	Neoplasm of the upper aerodigestive tract	No	Yes/no	Arch Otolaryngol Head Neck Surg 2001;127:870–876[16]
Mechanical and neuromyogenic OP dysphagia				
SWAL-QOL	Mechanical or neurologic OP dysphagia owing to multiple causes	Yes	Yes/no	Dysphagia 2000;15:122–133[17]
SSQ	Neuromyogenic or OP dysphagia with 3 months of stable symptoms	Yes	Yes/no	Gastroenterology 2000;118:678–687[18]
SWAL-CARE	Mechanical or neurologic OP dysphagia owing to multiple causes	No	Yes/no	Dysphagia 2002;17:97–114[19]
Achalasia				
MADS	Achalasia patients	No	Yes/yes	Am J Gastroenterol 2005;100:1668–1676[20]
Eckardt score[a]	Newly diagnosed achalasia patients undergoing pneumatic dilatation	No	Yes/no	Gastroenterology 1992;103:1732–1738[21]
Eosinophilic esophagitis				
DSQ	Adolescents and adults with EoE	No	Yes/yes	Aliment Pharmacol Ther 2013;38:634–642[22]
PEESS v2.0[a]	Pediatric patients with EoE	No	No/no	BMC Gastroenterol 2011;11:126[23]
EEsAI	Adults with EoE	No	No/no	Gastroenterology 2014;147:1255–1266.e21[24]
General dysphagia				
PROMIS-GI[a]	Multiple GI disorders and symptoms	No	Yes/no	Am J Gastroenterol 2014;109:1804–1814[1]
MDQ	Adults with dysphagia	No	No/no	Dis Esophagus 2007;20:202–205[25]
Heartburn				
GSAS[a]	Patients with GERD	Yes	Yes/yes	Dig Dis Sci 2001;46:1540–1549[26]
N-GSSIQ	Patients with GERD confirmed with pH monitoring, endoscopy, imaging, physician diagnosis, or PPI response with symptoms over previous 3 months	Yes	No/yes	Aliment Pharmacol Ther 2010;32:591–602[27]