International - Patient reported outcome of Achalasia Symptom Score

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Achalasia

- Absence of peristaltic contractions in the oesophagus (gullett)
- No relaxations of the distal part of the oesophagus at swallowing
- The food or liquid get stuck in the oesophagus
- The patient experience dysphagia, regurgitation of indigested food, chest pain
Esophageal Achalasia
Esophageal Achalasia
HRM: Normal Subject
HRM: Chicago Classification

**Pattern 1**
- no significant esophageal pressurization

**Pattern 2**
- rapidly propagated compartmentalized pressurization

**Pattern 3**
- rapidly propagated pressurization due to spastic contraction

*Pandolfino J Gastroenterol 2010*
Achalasia

- Rare disease (Incidence: 1-2.5 new patients/100,000 inhabitants/year)
- Prevalence (number of patients with the disease) is ten times higher
- Treatments are effective on symptoms, but do not eliminate the cause of the disease
- Chronic disease: many patients will experience more than one treatment in their life
How Achalasia Was Managed in UK
Data from HES* 2001-2012

*Hospital Episode Statistic

Markar S et al Br J Surg in press
How Achalasia Was Managed in UK Data from HES* 2001-2012

- 7487 pts (624 pts/yr)
- Pneumatic Dilatation: 4534 (60.6%)
- Myotomy: 1742 (23.3%)
- Botox: 1211 (16.1%)

*Hospital Episode Statistic

Markar S et al Br J Surg in press
What is a Patient-Reported outcome?

- PRO measures are defined 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.
- PRO measures are key elements for research on patient-centered outcomes.
Why We Need a PRO measure in Achalasia (I-PASS)

- Treatments for achalasia are aimed to relieve symptoms
- PRO measures are requested in RCT comparing different symptomatic treatments.
- No accepted definition for “symptomatic failure” in Achalasia
  - Eckardt score is not a PRO measure
  - Was never validated to assess the outcome of achalasia treatment
I-PASS Aims

• Create a *simple* patient reported outcome measure for achalasia to assess:
  – The severity of the disease
  – The effect of treatment

• Ideally, this new scale should be more comprehensive but have similarities to the Eckardt score

• Target population: adult achalasia patients
I-PASS Steps (a)

• Phase 1. 15 “experts” in achalasia select a number of items to assess severity and frequency of achalasia symptoms using a 3-phase Delphi Process

• Phase 2. five native English speakers translate each question in lay terms. A psychologist lead a Cognitive Interview with 5 patients to further refine the questionnaire and make it “patient friendly”
I-PASS Steps (b)

- Step 3. Pilot study on 50 naïve achalasia patients to assess the willingness to participate, the rate of acceptance, the satisfaction and the time needed to fill the questionnaire.

- Step 4. Translate the questionnaire in different languages and launch the validation study on a large number of patients (750 pts).
What we have achieved so far: I-PASS Lay-Term questionnaire

Pre-Treatment section

The first set of questions is about the severity of your trouble swallowing (dysphagia), that is when you feel the food or liquid that you swallow does not go through your swallowing tube smoothly or that gets stuck:

Do you have any trouble swallowing?

- No, I do not have any trouble swallowing
- I have regular trouble swallowing only certain food (rice, bread, apple, meat)
- I have regular trouble swallowing solid food (I need to drink to push food down)
- I have regular trouble swallowing liquid
- I have trouble swallowing both liquid and solid food,
What we have achieved so far: I-PASS Lay-Term questionnaire

The second set of questions is about the frequency of your trouble swallowing:

- I never experience any trouble swallowing
- I have trouble swallowing only once / twice a month
- I have more frequently trouble swallowing

If your swallowing problem (of any severity) is more frequent, please consider the last week:

- I have trouble swallowing once a week
- Every two to three days, or more
- At least once, every day
- At every meal
I-PASS Next steps

• Cognitive Interview
• Ethical permission for the pilot study (submitted)
• Pilot study: 50 naïve achalasia patients recruited in 3 Centers in London (IC, UCL, QMUL)
• Larger study: 750 patients (UK / EU grants)
I-PASS. What do we need from the UK achalasia patient support group?

- We need the patients opinion (5 volunteers for the cognitive interview)
- We need support for the pilot study (recommend patients to take part to the study)
- We need your help for asking for grants (UK / EU) for the larger study
- We need your help for recruiting treated patients for the validation study
I-PASS. Who is funding it?

- We received small grants from an Italian Charity (Morgagni foundation) dedicated to study esophageal diseases (5000 Euros)
- ALMA (an Italian achalasia patient supporting group) promised 1500 Euros
- ISDE (International Society for Diseases of the Esophagus) promised 3000 USD
I-PASS

Thank You for Your Attention

Q & A