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Patients-reported-outcome for oesophageal achalasia symptom score

Submission date
01/09/2020

Recruitment status
No longer recruiting

Registration date
14/09/2020

Overall study status
Completed

Last Edited
24/08/2023

Condition category
Digestive System

[X] Prospectively registered
[_] Protocol
[_] Statistical analysis plan
[_] Results
[_] Individual participant data
[_] Record updated in last year

Plain English summary of protocol

Background and study aims
Oesophageal Achalasia (OA) means that a person has difficulty swallowing food or liquids due to the muscles in their food-pipe not working properly. Patients may have chest pain, trouble swallowing food or liquids, weight loss, and may complain about food coming back into their mouth after eating. The incidence of OA is relatively low (1-2.5 new cases per 100.000 people per year), but because it is a chronic disease, it is estimated that total cases are around 10 patients per 100.000 people. OA is a chronic disease and currently, all treatment options are for the control of symptoms only and patients may require multiple interventions during their lifetime. There are currently three treatment options for OA: surgical or endoscopic division of the muscle between the oesophagus and the stomach, forceful pneumatic dilatation, and paralysis of the muscle by injecting botulinum toxin. The outcomes of these treatments are measured by symptom improvement; however, currently, there are no validated patient-reported outcome tools to measure achalasia symptoms.

With the help of seventeen international experts and cognitive interviews with patients, a new Patient Reported Outcome Tool has been created to quantify the symptoms in achalasia patients and to assess the efficacy of achalasia treatments. This is necessary for a period when different treatment options need to be compared in randomized controlled trials

Before embarking in a large study, a pilot study has been planned with the main aim of establishing the willingness of patients to fill in questionnaires related to their symptoms. This will allow the design of a larger study to test the capacity of the tool for detecting the success or failure of treatments. It is expected that the acceptance rate in the pilot study will be over 80%. If this target is achieved, a larger study to validate the I-PASS questionnaire will be created.

Who can participate?
Adult patients after their diagnosis has been confirmed by the usual tests for achalasia (High-resolution manometry, barium swallow, and UGI endoscopy). Patients younger than 18 years, not fluent in English, or with psychiatric or neurological disorders will be excluded. This study has been discussed and approved by the Association of Achalasia Patients in the UK.

What does the study involve?
Participants will be invited via an invitation letter, the study information leaflet, and consent form being posted to them. They will have a pre-paid envelope to return the documents. They will be provided with a contact number of the research team if they have any queries regarding the study. Participants will be given at least 1 month to decide whether on not they would like to participate in the study.

The patient will receive the I-PASS questionnaire and the short form 36 of the quality of life questionnaire once informed consent has been taken. The questionnaires will be posted to the participant with a prepaid envelope. Patients will fill in the two questionnaires individually (without any involvement of the nurse) however, the research team contact details will be available if the patient requires any further explanations. On completion of the questionnaires, the time needed to fill in the forms will be recorded by the participant. It is expected that the time needed to complete the questionnaires will be around 10 to 20 minutes.

The last questionnaire will then be filled to establish if the patient was satisfied with the questionnaires, if they understood all the questions, and whether they would be available to review the questionnaires again, after treatment.

What are the possible benefits and risks of participating?
There will be no direct or indirect benefit for the patients from their participation in the study, and there are not risks foreseen as a result of participation.

Where is the study run from?
Three NHS centers in London, Imperial College, University College, and Barts Health (UK)

When is the study starting and how long is it expected to run for?
From November 2019 to December 2022

Who is funding the study?
Fondazione Morgagni ONLUS (Italy)

Who is the main contact?
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Contact information

Type(s)
Scientific

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Additional identifiers

EudraCT/CTIS number
Nil known

IRAS number
267141

ClinicalTrials.gov number
Nil known

Secondary identifying numbers
IRAS 267141

Study information

Scientific Title
International patients-reported-outcome for Achalasia Symptom Score

Acronym
I-PASS

Study objectives
Oesophageal achalasia is a relatively rare disease characterized by the absence of opening of the cardia and the absence of peristaltic contractions along the oesophageal body. Consequently, when an achalasia patient swallows solid food or liquid, the bolus cannot pass into the stomach and he/she experiences dysphagia, regurgitation of undigested food into the mouth, or chest pain. It has been estimated that the incidence of OA is between 1 to 2.5 per 100.000 habitants per year, but the prevalence of the disease is ten times higher (10/100.000).
Most patients will require more than one treatment during their lifetime. The medical community treats the symptoms caused by OA (no etiological therapy exists) by lowering the resting pressure of the cardia. This is achieved by cutting the muscle, either during surgery or endoscopy or by forceful stretching of the muscle or by “poisoning” the muscle by injecting botulinum toxin into it. The efficacy of any of these treatment options is measured by symptom improvement, however, there are no validated patient-reported outcome questionnaires for achalasia symptoms. At present, the so-called “Eckardt score” is used, but this tool is not based on patient self-reporting, and therefore may be influenced by the interviewer and more importantly, it has never been properly validated. When we developed the International Guidelines for Oesophageal Achalasia, the lack of a validated tool that helps to define the success/failure of treatment was highlighted. A validated patient report outcome tool is also essential to compare the different treatment methods in randomized clinical trials and may facilitate the medical community accurately selecting the most appropriate therapy option.
We have created an international group of “achalasia experts” comprising of seventeen

gastroenterologists and surgeons from the UK, USA, Canada, Australia, Sweden, Norway, the Netherlands, Belgium, Germany, and Italy with the aim of creating this new tool (International Patient-reported outcome for Achalasia Symptom Scoring – I-PASS). Through a 3-round Delphi process, we have prepared a questionnaire, translated it into lay words, and refined it with cognitive interviews with 10 achalasia patients. Our hypothesis is that this new tool will allow to objectively measure the outcome of the different type of treatments of achalasia.

Ethics approval required
Old ethics approval format

Ethics approval(s)
Approved 21/07/2020, East Midlands - Nottingham 1 Research Ethics Committee (no address: +44 (0)207 104 8115; nottingham1.rec@hra.nhs.uk), ref: 20/EM/0167

Study design
Observational pilot study

Primary study design
Observational

Secondary study design
Cross sectional study

Study setting(s)
Hospital

Study type(s)
Diagnostic

Participant information sheet

Health condition(s) or problem(s) studied
Oesophageal achalasia

Interventions
Achalasia patients will be recruited in high-volume Upper Gastrointestinal (UGI) surgical and gastroenterological units. The recruitment of the patients will occur once the patients have completed their investigations (high-resolution manometry testing and barium swallow) and have a confirmed diagnosis of achalasia. UGI endoscopy will exclude any malignancies in the esophagus and the stomach.

Participants will be asked if they would like to participate in this research study via an invitation letter, the study information leaflet, and consent form being posted to them. They will have a pre-paid envelope to return the documents. They will be provided with a contact number of the research team if they have any queries regarding the study. Participants will be given at least 1 month to decide whether on not they would like to participate in the study. Once informed consent has been taken, questionnaires will be posted to the participant with a prepaid envelope.

The patient will then receive the I-PASS questionnaire and the short form 36 of the quality of life questionnaire. The I-PASS questionnaire has been developed through a 3-round Delphi process

and then translated into lay words and refined through cognitive interviews with 10 achalasia patients. Patients will fill in the two questionnaires individually (without any involvement of the nurse), however, the research team contact details will be available if the patient requires any further explanations. On completion of the questionnaires, the time needed to fill in the forms will be recorded by the participant. The last questionnaire will then be filled to establish if the patient was satisfied with the questionnaires, if they understood all the questions, and whether they would be available to review the questionnaires again, after treatment.

It is expected that the time needed to complete the questionnaires will be around 10 to 20 mins.

Intervention Type
Other

Primary outcome measure
The willingness of naïve achalasia patients to fill in questionnaires related to their symptoms measured by the proportion of the number of eligible patients invited to participate who complete the questionnaires

Secondary outcome measures
1. The time required for the patient to fill in the questionnaires recorded by patients on completion of the questionnaires
2. Patient satisfaction with the questionnaires measured using a follow-up questionnaire

Overall study start date
01/11/2019

Completion date
01/12/2022

Eligibility

Key inclusion criteria
1. Aged ≥18 years
2. Primary achalasia confirmed by High-Resolution manometry, Barium swallow and endoscopy
3. Not previously treated for achalasia

Participant type(s)
Patient

Age group
Adult

Lower age limit
18 Years

Sex
Both

Target number of participants
50

Key exclusion criteria
1. Not fluent in English
2. Diagnosis of neurological or cognitive impairment, and/or severe psychiatric disorder

Date of first enrolment
15/11/2020

Date of final enrolment
15/11/2022

Locations

Countries of recruitment
England

United Kingdom

Study participating centre
Imperial College NHS Trust
St Mary's Hospital
South Warf Road
London
United Kingdom
W2 1BL

Study participating centre
University College London Hospital NHS Foundation trust
250 Euston Road
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United Kingdom
NW1 2PG

Study participating centre
Barts Health NHS Trust
The Royal London Hospital
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Sponsor type
Hospital/treatment centre

Website
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ROR
https://ror.org/056ffv270

Funder(s)

Funder type
Charity

Funder Name
Fondazione Morgagni ONLUS - Fondazione Morgagni Charity

Results and Publications

Publication and dissemination plan
Planned publication of results in a high impact-factor journal

Intention to publish date
01/06/2023

Individual participant data (IPD) sharing plan
The datasets generated and/or analysed during the current study will be included in the subsequent results publication. Data collected will be anonymized and stored in password-protected Imperial College PC.

IPD sharing plan summary
Other