Peroral endoscopic myotomy (POEM) vs endoscopic balloon dilation (EBD) for the treatment of achalasia in children

Published: 21-06-2021
Last updated: 16-05-2025

To compare the efficacy of POEM to the efficacy of endoscopic pneumodilatation as initial treatment of symptomatic idiopathic achalasia in children. The primary outcome measure is the need of retreatment (i.e. EBD, LHM, POEM or intra-sphincteric...

Ethical review	Approved WMO
Status	Recruiting
Health condition type	Gastrointestinal motility and defaecation conditions
Study type	Interventional

Summary

ID

NL-OMON55648

Source
ToetsingOnline

Brief title
PEDPOEM

Condition

- Gastrointestinal motility and defaecation conditions

Synonym
Achalasia, Esophageal motility disorder

Research involving
Human

Sponsors and support

Primary sponsor: Academisch Medisch Centrum
Source(s) of monetary or material Support: Ministerie van OC&W,Stichting kindermotiliteit

Intervention

Keyword: Endoscopic, Myotomy, Pediatric achalasia, Pneumodilation

Outcome measures

Primary outcome

Primary endpoint is treatment success, defined as a Eckardt score of 3 or less in the absence of the need for endoscopic or surgical retreatment and the absence of severe complications associated with treatment. The primary endpoint is measured after 1 year.

Secondary outcome

- Quality of life and achalasia-specific quality of life

- Stasis in the oesophagus, measured with a timed barium oesophagogram

- Presence of reflux symptoms, reflux oesophagitis and excessive oesophageal acid exposure

- Lower oesophageal sphincter pressure and integrative relaxation pressure (IRP4), as measured with high-resolution impedance manometry

- Complications of the treatment, defined as any unwanted events that arise following treatment and/or that are secondary to the treatment. Complications are classified as *severe* when these result in admission > 24 hours or prolongation of an already planned admission of >24 hours, admission to a medium or intensive care unit, additional endoscopic procedures, or blood transfusion or death. Other complications are classified as *mild*.

Study description

Background summary

Achalasia is a rare motility disorder of the esophagus that is characterized by aperistalsis of the oesophageal body and dysrelaxation of the lower oesophageal sphincter. Achalasia is caused by progressive destruction and degeneration of the neurons in the myenteric plexus. This leads to subsequent retention of food and saliva in the oesophagus, resulting in the typical symptoms of achalasia such as dysphagia, chest pain, regurgitation of undigested food and weight loss. Treatment of achalasia is focused on symptom relief, which is obtained by destroying the occluding function of the spastic lower oesophageal sphincter. Treatment options are endoscopic dilatation by a pneumatic balloon, the golden standard, or Heller myotomie a surgical treatment performed by laparoscopy. Endoscopic pneumodilatation can be complicated by a perforation and there is a relative high chance of symptom recurrence which requires subsequent treatment sessions. The surgical treatment can also be associated with severe complications, like a perforation, and is more invasive. Currently endoscopic pneumodilatation is the first choice of treatment in patients with achalasia and surgical myotomy is generally performed in case of symptom recurrence after initial pneumodilatation. The recent developments on minimal invasive surgical techniques has led to the development of per-oral endoscopic submucosal myotomy (POEM) for the treatment of achalasia. The first results of the POEM are very positive and suggest that this treatment is better than the endoscopic pneumodilation.

Study objective

To compare the efficacy of POEM to the efficacy of endoscopic pneumodilatation as initial treatment of symptomatic idiopathic achalasia in children. The primary outcome measure is the need of retreatment (i.e. EBD, LHM, POEM or intra-sphincteric botox injection) due to persisting symptoms (Eckardt score > 3) with evidence of recurrence on barium swallow and/or HRM at 12 months follow-up.

Study design

Multicenter randomized clinical trial.

Intervention

Study subjects undergo a POEM or endoscopic pneumodilatation

Per-oral endoscopic submucosal myotomy (POEM): The POEM technique is entirely endoscopic. Using an endoscopic knife, an entry to the submucosal space is made in the oesophagus and after creating a submucosal tunnel towards the lower oesophageal sphincter the circular muscle layers are cut. At the end of the procedure the mucosal opening is closed with clips.

Endoscopic pneumodilatation: Endoscopic dilatation of the lower oesophageal sphincter is first performed by a Rigiflex balloon of 30mm and a second dilatation is performed two weeks later with a Rigiflex balloon of 35mm. A third dilatation is performed if the Eckardt symptomscore is above 3, within 6 months after the first dilatation or if barium esophagram and/or HRM are suggestive for relapse.

Study burden and risks

For this study patients with achalasie will be treated with per-oral endoscopic myotomy (POEM) or endoscopic pneumodilatation Both procedures are associated with risks, including a bleeding during or after treatment, a perforation of the oesopahgus or stomach during the treatment and an infection after treatment. These complications can be solved during the endoscopy in most cases. Refer to protocol 9.4 for more details.

To determine effectiveness, patients are followed for a period of 1 year. Patients need to fill out questionnaires at 3 and 6 month post-operative and at 1 year follow-up. Furthermore they need to undergo a gastroscopy, a timed barium oesophagogram, high resolution manometry and a 24 hour pH-impedance monitoring. The barium-esophagram is the only invasive investigation duringe follow up, that is not performed during standard clinical care, yet is needed to evaluate treatment efficacy. After initial procedure study subjects need to visit the outpatient clinic three times for follow-up.

The first results of the POEM are very promising and suggest that this treatment is better than the endoscopic pneumodilation, the gold standard. The risks of both procedures are likely very similar. Furthermore patients that are not participating in the trial will undergo a treatment anyway, in most cases this will be endoscopic pneumodilatation.

Contacts

Public
Academisch Medisch Centrum

Meibergdreef 9
Amsterdam 1100DD
NL
Scientific
Academisch Medisch Centrum

Meibergdreef 9
Amsterdam 1100DD

NL

Trial sites

Listed location countries

Netherlands

Eligibility criteria

Age
Adolescents (12-15 years)
Adolescents (16-17 years)
Children (2-11 years)

Inclusion criteria

Newly diagnosed achalasia patients aged 7 - 18 years old with
- Eckardt score > 3;
- presence of a high resolution manometry pattern consistent with achalasia type 1 or 2 according to the Chicago classification (CC) V3.0 criteria
- barium esophagram suggestive of achalasia

Exclusion criteria

- Previous endoscopic or surgical treatment for achalasia
- Previous surgery of the upper gastrointestinal tract
- Known coagulopathy
- Liver cirrhosis and/or esophageal varices
- Known grade >=B esophagitis
- Barrett*s esophagus
- Known pregnancy at time of treatment
- Stricture of the esophagus
- Presence of malignant or premalignant esophageal lesions
- Hiatal hernia > 1cm
- Extensive, tortuous dilatation (>7cm luminal diameter, S shape) of the esophagus
- Barium esophagram suggestive for other pathologies

Study design

Design

Study type:	Interventional
Intervention model:	Parallel
Allocation:	Randomized controlled trial
Masking:	Open (masking not used)
Control:	Active
Primary purpose:	Treatment

Recruitment

NL

Recruitment status:	Recruiting
Start date (anticipated):	09-02-2022
Enrollment:	30
Type:	Actual

Ethics review

Approved WMO Date:	21-06-2021
Application type:	First submission
Review commission:	METC Amsterdam UMC
Approved WMO Date:	08-06-2022
Application type:	Amendment
Review commission:	METC Amsterdam UMC
Approved WMO Date:	09-06-2022
Application type:	Amendment
Review commission:	METC Amsterdam UMC
Approved WMO Date:	13-06-2024
Application type:	Amendment

Review commission:	MEC Academisch Medisch Centrum (Amsterdam) Kamer G4-214 Postbus 22660 1100 DD Amsterdam 020 566 7389 mecamc@amsterdamumc.nl

Study registrations

Followed up by the following (possibly more current) registration

No registrations found.

Other (possibly less up-to-date) registrations in this register

No registrations found.

In other registers

Register	ID
CCMO	NL68967.018.20