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Cover Page Footnote

The author would like to acknowledge Vishal Varma for his unwavering support.

Pneumatic balloon dilation versus myotomy for the treatment of achalasia

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ABSTRACT A critical appraisal and clinical application of Moonen A, Annese V, Belmans A, et al. Long-term results of the European achalasia trial: A multicentre randomised controlled trial comparing pneumatic dilation versus laparoscopic Heller myotomy. *Gut*. 2016 May;65(5):732-9. doi: [10.1136/gutjnl-2015-310602](https://doi.org/10.1136/gutjnl-2015-310602).

Keywords: *Heller myotomy, balloon dilation, achalasia*

Clinical Context

The patient is an 18-year-old African American female with a six month history of difficulty swallowing, both liquids and solids. The dysphagia has gotten progressively worse, to the point that over the last two months she has been unable to swallow any liquids or solids, including water, without vomiting the contents back up within minutes. She has thus lost nearly 60 lbs over 6 months. She also had intermittent chest pain associated with the vomiting. She had two esophagogastroduodenoscopies (EGDs) since symptom onset that were suggestive of achalasia, but had poor follow-up and ultimately was admitted to the hospital due to malnutrition. Inpatient esophageal manometry confirmed the diagnosis of achalasia.

A consult was placed to general surgery, and the surgery team indicated that the patient was a candidate for surgical treatment via Heller myotomy. Major surgery is a large decision for any patient, and especially so for a young adult, so other modalities were considered. Achalasia is also commonly treated with pneumatic balloon dilation of the lower esophageal sphincter, which was offered by the gastroenterology consult service.

As a young female with most of her life ahead of her, our patient was interested in long-term solutions that would hopefully limit recurrent symptoms and the need for more procedures in the future.

Clinical Question

Does Heller myotomy for treatment of achalasia provide longer post-treatment periods without recurrent symptoms versus pneumatic balloon dilation, and reduce the need for future treatments, including subsequent balloon dilations?

Research Article

Moonen A, Annese V, Belmans A, et al. Long-term results of the European achalasia trial: A multicentre randomised controlled trial comparing pneumatic dilation versus laparoscopic Heller myotomy. *Gut*. 2016 May;65(5):732-9. doi: [10.1136/gutjnl-2015-310602](https://doi.org/10.1136/gutjnl-2015-310602)

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Related Literature

The PubMed research database was queried for literature appraisal. Search terms included “achalasia,” “balloon dilation,” “Heller myotomy,” “esophageal dilation,” and “myotomy”; “dilatation” was substituted for “dilation” where appropriate; permutations of these terms with modifiers such as “versus” and “compared” were used to yield a comprehensive search of the literature. This search revealed over 50 manuscripts potentially relevant to the question at hand. Search results were reviewed based on article abstracts to determine which articles were pertinent to the clinical question. Several meta-analyses relevant to this topic were reviewed for additional relevant citations.^{2,3} Secondarily, the UpToDate article related to treatment of achalasia was perused to ensure a comprehensive literature review. Using the Advanced Search function in PubMed with search terms ‘(achalasia[Title/Abstract]) AND therapy[MeSH Terms]’ filtered by RCT and Humans yielded 39 papers which were reviewed for relevance, finding one additional paper that was evaluated.⁹

The Moonen study demonstrates many desirable aspects for application to the clinical question. First of all, the prospective randomized design provides the most robust setting for limiting bias and maximizing utility of results. The study includes patients of both sexes, 18 years and older treated for idiopathic achalasia, which permits application to our patient. Patients were randomized to either Heller myotomy or pneumatic balloon dilation treatment, which was our clinical question for how to best treat this patient. 1- and 5-year follow-up data was recorded, which is relatively lengthy follow-up for a study such as this and helps answer our question about long-term outcomes and retreatment needs. Furthermore, this study had one of the largest sample sizes for a randomized controlled study of this treatment dichotomy.¹

In reviewing other literature on the subject, the study by Persson et al. was a randomized controlled prospective trial similar to the above study by Moonen. Patients randomized to the Heller myotomy group demonstrated lower rates of treatment failure compared to those in the pneumatic dilation group.⁴ This study was limited by a smaller sample size (n= 53) and did not achieve 5-year follow-up for most of the patients in the study, making it less apt to answer our clinical question.

Chrystojaj’s group also published a randomized prospective study relevant to our appraisal. Both groups experienced similar initial treatment success and quality of life at 1-year follow-up, with the only difference being fewer retreatments in the Heller myotomy group.⁵ This study was also limited by a smaller sample size (n=50) and was without long-term follow-up.

The Tabola study was a prospective study following two cohorts: one treated with balloon dilation and the other with myotomy. Patients in both groups had a similar mean time to recurrence of symptoms, however patients in the myotomy group had higher scores on surveys administered to capture symptom improvement after the respective procedures.⁶ The study population included few patients under 30 years old and no patients under 20 years old, which limits application to our patient. Furthermore, there was only a 2-year follow-up, which again does not address our question about longer-term outcomes.

Vela et al. performed a cross-sectional follow-up study on patients treated for achalasia with either Heller myotomy or pneumatic dilation. Patients treated with dilation were more likely to require subsequent treatment at both the 6-month and 6-year marks.⁷ This study is limited by its retrospective design, but does provide useful long-term follow-up data. Of note, this study is also a bit more dated than most studies discussed here.

Zagory’s group offered a retrospective study of outcomes in children treated for achalasia. Children treated with Heller myotomy had a higher rate of long-term symptom relief without requiring further treatment while many of those in the dilation group required a subsequent dilation and/or myotomy due to recurrent symptoms.⁸ This study was limited by sample size (n=23). The patient ages were also younger than the patient in our appraisal, however it is worth noting that the mean age for the myotomy group was 15.6, which is not too far removed from 18 years old.

Of note, there exist multiple papers authored by Boeckxstaens et al. These studies are the same data pool as the study being appraised, both utilizing the “European Achalasia Trial”. Importantly, the initial study followed patients only through 2 years, while we are interested in longer-term outcomes due to the desires of our patient in question. These initial studies are obviously of use, but the study we chose provides the longest follow-up of this useful dataset.



Based on the shortcomings pointed out in the above studies, including many with smaller sample sizes and others with suboptimal study design, the study by Moonen's group was chosen for appraisal. This study includes one of the largest sample sizes for a prospective randomized clinical trial, making it a highly valuable piece of medical literature.

Critical Appraisal

Based upon the SORT taxonomy¹⁰, this study demonstrates Level 1 evidence; the study is a prospective randomized trial, which is an ideal study design. The goal of this study was to compare two treatments which are both argued to be standard of care, depending on other literature and which physician's opinion is elicited, so the study was intentionally not designed to have a placebo control. While there are some useful results in the subgroup and post-hoc analyses, the two treatments did not significantly vary in their immediate symptom reduction or long-term efficacy, so overall effect size is not commented on here.

The study design includes patients from 14 hospitals across 5 European countries. There is no table or inline comment on racial distribution of the patients enrolled in the study, so this study cannot be used to apply directly to different racial groups. This may be due to the largely Caucasian preponderance of the population in Europe; however reporting of the numbers of each race included in the study would be useful and simple for the authors. 40% of the study population was under 40 years of age and nearly half of the population (47%) was female. This is a fair representation of young females in this study, making our patient sufficiently similar to the study population for comparison. Randomization was performed via a computer algorithm and patients were randomized to groups <40 years old and ≥ 40 years old, which is relevant for our young patient. Randomization was sufficient based on the baseline characteristics table. The two groups were treated equally other than the treatment administered.

Patients were recruited to the study only upon receiving a diagnosis of achalasia based upon esophageal manometry studies, the gold standard for diagnosis.¹¹ There were no sham scars or wounds made in the pneumatic dilation group, so after treatment patients became aware of which group they were randomized to. This allows for some possible performance bias in future follow-up questionnaires, however sham operations are not an aspect of current medical research and thus this study was blinded as optimally as could be expected.

Heller myotomy was performed laparoscopically; the perioperative course and treatment of the patients was not directly commented on, and theoretically could play a role in immediate post-operative recovery, however there were no reported events related to anesthesia or other perioperative factors, so it does not appear to have an effect on the study or outcomes.

In disparity to some other studies, the requirement for subsequent pneumatic dilations was not considered a failure modality in treatment with dilation. Many studies consider this a failure of treatment, while plenty of others do not, so this seems to be a choice of the authors and does not invalidate the study overall. Specifically, our patient had issues with follow-up and was relatively young, so this is relevant to her, as requiring subsequent dilations years down the road could cause a similar spiral of severe malnutrition that occurred during this initial bout. To improve this, the authors could perform a separate analysis in which requiring a second balloon dilation is registered as a failure of treatment, and provide these results for comparison.

Two hundred eighteen patients were enrolled, four excluded before randomization due to diagnosis of pseudoachalasia. 105 patients were originally randomized to laparoscopic Heller myotomy (LHM), and 109 randomized to pneumatic dilatation (PD). Thirteen patients from the PD group were excluded from analysis due to treatment with an outdated protocol not consistent with study design. Two primary PD patients would elect LHM for subsequent treatment and were switched to the LHM group for analysis; two more PD patients underwent re-dilatation that was outside of the study protocol, and they were not included; seven PD patients had progressive symptoms but refused subsequent dilatation and were excluded, leaving 85 patients. Fifty Seven out of 85 patients in this PD group completed 5-year follow-up. For the LHM group, the 105 originally randomized patients mentioned above increased to 107 with the addition of the two patients who underwent LHM after pneumatic dilatation. Seventy one out of 107 patients in this LHM group completed 5-year follow-up for analysis.

Examining the results using the per-protocol groups as outlined above, there were significantly better treatment outcomes at 5-year follow-up for patients 40 years and older who underwent pneumatic dilatation with $p=0.01$. For patients under 40 years of age, there was no difference in outcomes between the two treatments at 5-year follow-up with $p=0.89$, a more important measure for our young patient and one consistent with the overall equivocal nature of the literature. Of note, 25% of patients in the PD group did



require a repeat dilation, however this was not considered a treatment failure in the context of this study but does bear relevance to our young patient who will be living with this condition for the rest of her life.

For both treatment groups, a quantitative symptom-scoring questionnaire (Eckhardt score) was used for pre-treatment and follow-up assessment of symptom severity. This was an effective and relatively standardized way of capturing severity of disease and helps make long-term follow-up more reliable and interpretable since it is the same system used before any treatment. While not formally administered to our patient, she did have an Eckhardt score >3 based on her symptoms, making the study results applicable to her from this perspective.

Primary outcome was reported as symptom success based upon an Eckhardt score of 3 or less at 1-year follow-up. Post-treatment lower esophageal sphincter pressure, quality of life based on a standardized questionnaire and rate of complications were also reported for each treatment. The complications reported are specific to the procedures, including esophageal tears, lacerations or development of reflux-like symptoms. While these are the most directly pertinent, it would have been useful to have a brief discussion of other common perioperative side effects, such as an adverse reaction to sedation or surgical wound site infections. If neither of these happened, a simple sentence stating so should have been included, as the modality and strength of sedation was likely different between the two groups, one being invasive surgery and the other being endoscopic in nature. Clinical significance was adequately considered in that the need for further treatment and quality of life were addressed, which was the main purpose of this study of long-term outcomes. Statistical adequacy was rigorously considered at appropriate power levels and was done on both an intention-to-treat and per-protocol basis. While intention-to-treat is the stronger methodology, it was ideal to have both methods utilized for a more comprehensive look at the data.

There was no funding bias directly related to this study. Two of the investigators reported receiving funding either personally or institutionally from various pharmaceutical and other medical companies, however in review of the nature of the compensation, none of the funds had any relationship to the study at hand.

There is no publication bias. The study reports being registered prospectively and the study protocol did not deviate from the initial proposition according to the manuscript. There is no apparent missing data from what was set out in the methods.

Clinical Application

The patient is an 18-year-old African American female with poor follow-up who presented for severe malnutrition and weight loss secondary to idiopathic achalasia. She and her family preferred the most definitive treatment option in hopes of achieving a cure, in other words not requiring further or repeat treatment years down the line. Our patient does meet the inclusion criteria outlined in the appraised study.

In view of the patient's desires and the study appraised above, Heller myotomy is the more appropriate option for our patient. Specifically, the results indicate that young age (<40 years) was a risk factor for requiring subsequent balloon dilation after undergoing balloon dilation as the primary treatment, whereas being young did not correlate with poorer outcomes in the Heller myotomy group. Furthermore, the appraised study allowed for up to three balloon dilations before considering this modality a failure; our patient viewed even a single subsequent treatment as a failure, making her criteria more stringent and in favor of the Heller myotomy, which in our study often amounted to the same outcomes as two or even three balloon dilations.

Internal validity of the study was demonstrated appropriately based on their parameters. When comparing Heller myotomy to up to three balloon dilations, the results are similar for 1-, 2- and 5-year outcomes in terms of symptom reduction and quality of life. Without considering the exact parameters and wishes of our patient as above, either treatment can be chosen with confidence and without worry of deviating from the standard of care.

As noted above, our patient was a better candidate for Heller myotomy in hopes of not requiring further treatments, as was relatively common with balloon dilation. In this way, the cost to her time in the hospital and for recovery was more "up front" with the single Heller myotomy versus potentially having to undergo as many as three separate procedures and the time cost associated with each. Furthermore, the poor follow-up demonstrated



in the initial bout of disease may be a running theme, which would limit the patient returning for necessary subsequent treatments.

Benefits of applying this research to our patient include decreased need for future or repetitive treatments of a chronic disease, decreased time lost associated with future treatments and no detriment in overall outcomes compared to balloon dilation. Harms include those associated with major surgery, including those related to general anesthesia, wound healing and hospital-associated infection which may not be as strong in a more outpatient based pneumatic dilation setting.

Learning points:

1. Heller myotomy and pneumatic balloon dilation are both appropriate treatments well within the standard of care for achalasia with comparable long-term outcomes including symptom relief and quality of life.
2. Younger patients with a long-term chronic disease may often express a stronger desire for a “curative” treatment, and this requires sufficient research on treatment options to ensure that the chosen option is appropriate and not putting the patient in harm’s way.
3. Despite increasingly abundant high quality research, there is still no definitive cure for achalasia, and it should be viewed as a chronic disease which will need continued reevaluation and likely treatment to best help patients.

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