

# Core outcome set for pulmonary rehabilitation of patients with COPD

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# **Impact section**

## Impact section

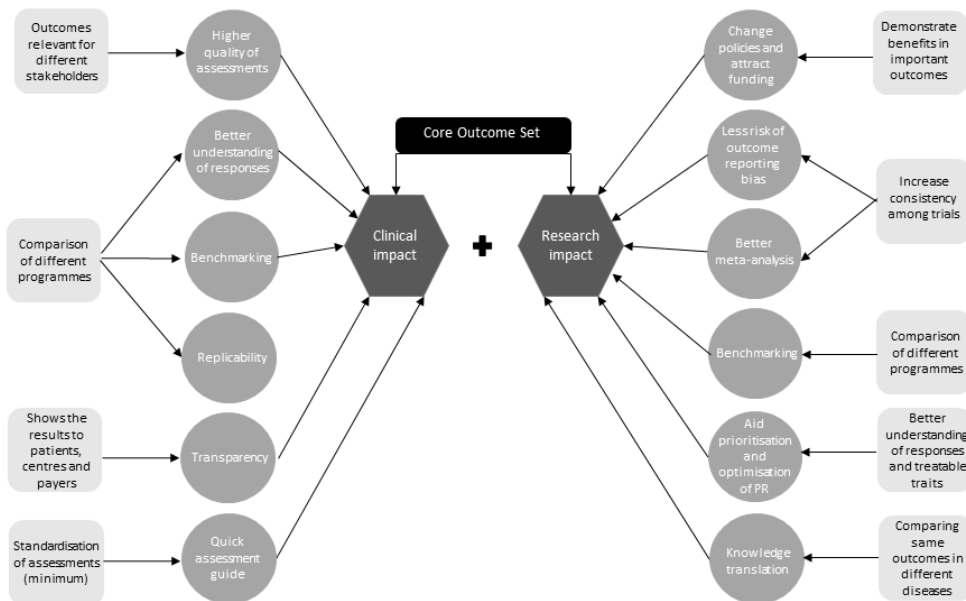
This thesis provided various findings that can have a significant impact beyond the research field. This section reflects on the scientific and social impacts of this thesis considering four paradigms: 1) Research – What is the main aim of the thesis and the main results and conclusions; 2) Relevance – What is the (potential) contribution of the findings of the thesis to the scientific and social sectors?; 3) Target groups – To whom and why are the scientific results relevant/favourable; and 4) Activities – How can the target groups be involved in and informed of the findings, so the knowledge can be used in the future?

### Research and relevance

The main aim of this thesis was to develop a COS. We found that nine outcomes are crucial to be measured from the perspectives of different stakeholders and should therefore be used hereafter in PR trials and clinical practice. This thesis also contributed to the knowledge in the field of treatable traits, by identifying a robust marker of functional impairment and showing that a treatable trait strategy should be further explored, since patients who exhibit treatable traits at baseline are those who respond better.

A COS for PR in patients with COPD might result in direct clinical and research benefits (**Figure 1**). Clinically, including outcomes relevant to different stakeholders might improve the quality of patient's assessments, it may enhance the understanding of patients' responses, enable benchmarking and replicability of best practices (by comparing the same outcomes between different programmes/populations), and by showing the results to patients, centres, and payers we can improve transparency and ensure trust among all parties. A COS may also help healthcare professionals to implement PR programmes, with a quick assessment guide.

Considering the research field, the developed COS might generate consistency among trials, whilst lessening the risk of outcome reporting bias, thus enabling the comparison of different studies across distinct settings. Moreover, the standardisation of outcomes achieved through a COS, may help to change policies regarding PR, by consistently showing the benefits of the intervention and therefore attracting funding to improve its access. Using the COS in research studies may also enable benchmarking PR (detecting the best model). Additionally, by enhancing our understanding of responders and non-responders to PR and of treatable traits, a COS might indirectly help to prioritise patients to this scarce intervention and promote its personalisation. Finally, the core outcomes can be compared across different diseases, to better understand the effectiveness of rehabilitation services for different groups of patients, and therefore translate knowledge from the best practices to those in need.



**Figure 1.** Potential impact of the core outcome set for pulmonary rehabilitation of people with chronic obstructive pulmonary disease (COPD). TT: treatable traits; PR: pulmonary rehabilitation.

## Target groups

Results of this thesis are of interest to a wide community of patients, researchers, clinicians, regulators, and other professionals involved in outcome measurement and confirm the beneficial effects of PR for patients with COPD.

Patients and patient organisations should be aware of this COS, so they can identify centres with the best practices, know their progress with PR and what is expected. Additionally, it may enable peer comparison in a positive competitive way and easy transfer of data from centre to centre when patients are referred to other PR programmes. Furthermore, patients and patient organisations might encourage PR practices to adopt this COS.

For clinicians, this COS might improve their efficiency in assessment and health record registration, by having a structured set of assessments to perform and

results to register. In fact, in the future it could be of value to have the COS embedded in electronic health registry systems, to improve the level of information on patients' records, facilitate data collection, transfer between centres and analysis of big data for multiple centres, e.g., within a country.

Researchers planning studies with PR as an intervention should now consider the full use of this COS to assess the effectiveness of their interventions and use one of the core outcomes as a primary endpoint in their analysis, so their results can be later combined in meta-analysis.

This thesis will greatly benefit systematic reviewers. If this COS has high uptake by trialists, systematic reviewers will be able to perform bigger data pools and meta-analysis, and therefore increase the strength of certainty about the evidence in the field.

Professional bodies and guideline developers/policy makers will also benefit from the work of this thesis, as it can serve as a robust base to drive recommendations on outcome measurement in PR and therefore foster higher quality in PR programmes worldwide.

Research funders might also benefit from this work, as they can encourage the use of more robust designs in studies during the application process and therefore fund studies who are using the COS which might have a lesser risk of outcome reporting bias. Similarly, trial registries and journals could incorporate in the submission form a question related to the use of a COS and therefore promote outcome reporting transparency.

Finally, this COS might also be of value to health technology assessment and audit agencies. For health technology assessment, the COS can be used to test different devices using the same outcomes (especially important for tele-rehabilitation) which can enable determining their access and pricing. For audit agencies, this COS can be part of an iterative process of quality improvement of PR,

using the core outcomes to ascertain effectiveness of different centres and assign best practices, in conjunction with other indicators, such as process and structure measures.

### **Activities performed and planned**

Many activities have been conducted to ensure the dissemination of results of this thesis. Firstly, results have been published in international peer-reviewed journals and have been presented in international and national scientific meetings.

Studies have also gain interest in social media and have been shared by different professional groups, such as the PR assembly of the American Thoracic Society, and the Group 1.02 “Rehabilitation and Chronic Care” of the European Respiratory Society.

Additionally, results have been shared with multiple stakeholders (patients, clinicians) and professional bodies throughout the COS process and have gained positive feedback and further attention from the general public. Results were also reported in a Portuguese news outlet *My Pneumologia*.

Nonetheless, many activities still need to take place in the future. The final results of this thesis (**Chapter 7**) will be disseminated in national and international congresses, with all stakeholders involved in the studies (**Chapter 6 and 7**), professional associations (local and international), patient organisations, research funders and health technology assessment and audit bodies. Finally, after the “how to measure” phase is established, support to healthcare professionals will be provided through training courses and online material on how to use the core measurement instruments.