Developing and validating a patient-reported outcome instrument to monitor symptom management and its influencing factors in acute phases in patients living with cystic fibrosis

Abstract

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Introduction: The main goals of cystic fibrosis (CF) management are to slow disease progression, to prevent pulmonary exacerbations, to provide symptom relief and to improve quality of life. To meet these goals, patients have to adhere to a complex and time-consuming daily medical regimen. Despite preventative measures, pulmonary exacerbations are frequent, with approximately 2-3 sequences per year in adults. In recent decades, the number of home intravenous therapies for the treatment of exacerbation has risen steadily. Evidence indicates that home therapy does not harm individuals, but may positively or negatively impact quality of life. One reason may be that managing exacerbation and intravenous therapy at home is a highly demanding process for patients, with the additional therapy compounding an already challenging symptom and treatment burden. Furthermore, evidence suggests that complexity of treatment affects adherence, producing negative clinical outcomes. While there is some evidence regarding patients' symptom experiences, little is known about patients' experiences with symptom management and treatment burden during exacerbation. This is in spite of the focus on assessments of patients' symptom experience in research and the clinical setting in recent decades. Patient reported outcome (PRO) measures aim at assessing interventions, monitoring changes, responding to treatment, and providing a basis for clinical decision making. To date, three PRO instruments focus on long-term quality of life in patients with CF and two PRO instruments focus on symptom experience in general, but no instrument exists to monitor symptom management and its influencing factors in acute phases of exacerbation. As exacerbations are a heavy burden on these patients, there is a need for an instrument to assess treatment and symptom experience during exacerbation. Such an instrument is needed as basis for shared decision making and to evaluate the effectiveness of interventions such as patient education.

Aim: The overall aim is to develop and validate a PRO instrument for measuring symptom in acute phases of CF patients. The instrument should provide a basis for evaluating interventions such as patient education as well as a basis for shared-decision making in future episodes of exacerbation. The instrument must be sensitive to change, cover all relevant factors influencing symptom management in acute phases and be appropriate for daily use.

Methodology: For this project, a mixed-method approach will be applied. The Revised Symptom Management Conceptual Model of Dodd and colleagues will provide the conceptual framework. The development of the PRO instrument will follow FDA Guidance. In the first step, the literature regarding possible theoretical frameworks and instruments will be reviewed. Next, patients' experience of symptom management during exacerbation will be investigated with a qualitative approach, using framework analysis procedures. A draft of the instrument will be developed. Content validity will be established with experts' rating, followed by a pilot-testing in 3-5 patients using cognitive debriefing techniques. Construct validity, criterion validity and feasibility will be examined in a sample of approximately 50 patients.