

Quality of Life Assessment in Sarcoidosis



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KEYWORDS

• Sarcoidosis • Health-related quality of life • Patient-reported outcomes • Symptoms • Function

KEY POINTS

- Health-related quality of life (HRQL) is important in assessing sarcoidosis; quality of life issues are important in determining the need for treatment.
- HRQL can be assessed accurately using established patient-reported outcome measures (PROs).
- Recently, several sarcoidosis-specific PROs assessing HRQL have been developed for clinical use.

INTRODUCTION

Health-related quality of life (HRQL) has become an important aspect of patient evaluation. This may be especially true with sarcoidosis, because the disease is rarely fatal and the physiologic manifestations of the disease are often mild. Over the last few decades, the assessment of HRQL has become more rigorous, as patient-reported outcome measures (PROs) have been developed that have the capacity to quantify this patient assessment. In this article, we describe the importance, relevance, general methodology of construction, and application of PROs and HRQL assessment in sarcoidosis.

THE IMPORTANCE OF QUALITY OF LIFE ASSESSMENT IN SARCOIDOSIS

Sarcoidosis may involve any organ. The disease course is highly variable, ranging from an asymptomatic state to a progressive condition that may, occasionally, be life threatening. The pathologic hallmark of sarcoidosis is the granuloma that may resolve spontaneously or with ant sarcoidosis therapy. Approximately 10% to 30% of sarcoidosis patients develop significant fibrosis that may result in permanent organ injury.¹

Regardless of the clinical course, the decision to treat sarcoidosis is usually based on the presence of 1 or both of the following 2 conditions resulting from the disease: a situation of potential danger to the patient or a significant worsening of the patient's quality of life. Of these 2 treatment indications, sarcoidosis-induced significant worsening of quality of life is, far and away, the more common.

Despite the importance of the impact of sarcoidosis on quality of life, clinicians often fail to give this issue adequate importance and base treatment decisions on other factors. This situation is depicted in **Fig. 1** in relation to pulmonary sarcoidosis.² Sarcoidosis causes granulomatous inflammation. This granulomatous inflammation may not cause a significant physiologic derangement or result in a significant decrease in quality of life. Even when the granulomatous inflammation of pulmonary sarcoidosis does cause physiologic abnormalities, they are often minor^{3,4} and do not always lead to appreciable symptoms.⁴ In these situations, unless these minor physiologic derangements are associated with dangerous consequences, there is not an adequate indication for treatment. Nonetheless, clinicians often initiate ant sarcoidosis treatment based on the presence of granulomatous inflammation (eg, increased

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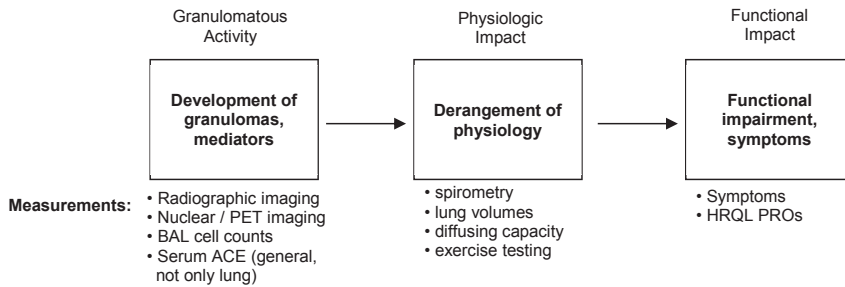


Fig. 1. Relationship of granulomatous inflammation, physiologic derangements, and HRQL impairment in pulmonary sarcoidosis. ACE, angiotensin-converting enzyme; BAL, bronchoalveolar lavage; HRQL, health-related quality of life; PRO, patient-reported outcome measure. (Adapted from Judson MA. The treatment of pulmonary sarcoidosis. *Respir Med* 2012;106(10):1353; with permission.)

serum angiotensin-converting enzyme levels, nodularity on chest imaging) or on the basis of physiologic change (eg, decrements in spirometry) when the patient's quality of life is not affected appreciably. Previous studies have shown that sarcoidosis experts are relatively poor judges of the impact of the disease on the patient's quality of life.⁵

HRQL is a multidimensional construct composed of several domains, including physical, social, mental/emotional, cognitive, and spiritual, and refers to the extent to which 1 or several of these domains are affected by a medical condition and/or its treatment.⁶ Serious consideration of HRQL results in a paradigm shift in patient care that has several significant consequences. First, attention to HRQL actively engages the patient in his or her health care, because patient input is required to make such assessments. Second, clinicians are obligated to focus on clinical information beyond objective laboratory data in considering HRQL. Such a process tends to redirect caregiver focus toward the real patient and away from the "iPatient," the virtual construct of the patient consisting of the patient's electronic medical record where patient assessment primarily involves interacting with a computer screen and not the real patient.⁷ Third, giving major consideration to HRQL allows the clinician and the patient to work toward a common goal of maximizing the patient's sense of health as opposed to maximizing laboratory test results that, in the case of sarcoidosis, are very often discordant.⁵

THE ASSESSMENT OF HEALTH-RELATED QUALITY OF LIFE

The assessment of HRQL does not necessarily mandate the use of objective measures. Clinicians often use qualitative measures by asking the patient to describe his or her symptoms and inquiring about how those symptoms affect their ability to

function and their quality of life. Although such an approach is often reliable when therapy results in changes of great magnitude that lead to substantial improvements in HRQL, such an approach may be less reliable in detecting small but significant changes in HRQL. In the case of pulmonary sarcoidosis, the sarcoidosis-induced physiologic decrements are often relatively minor,^{3,4} suggesting that a qualitative approach may be inadequate. Furthermore, in the case of assessing an intervention in a clinical trial, it is problematic to apply such qualitative assessments. Finally, as mentioned, clinical sarcoidosis experts only have a fair agreement with patients in the assessment of perceived sarcoidosis symptoms.⁵

HRQL can be quantitatively assessed using PROs. A PRO is any report coming directly from patients, without interpretation by physicians or others, about how they function or feel in relation to a health condition and its therapy.^{8,9} PROs have been used to evaluate general HRQL as well as specific symptoms experienced by the patient. Furthermore, PROs may be scaled and validated so that PRO measures accurately quantify the patient's perception of a specific symptom or state. PROs have become a standard measure used in clinical trials, because some effects of a health condition and its therapy are known only to patients. Properly developed and evaluated PRO instruments have the potential to provide more accurate measurements of the effects of medical therapies, thereby increasing the efficiency of clinical trials that attempt to measure the meaningful treatment benefits.⁹

PROs have become a standard measure used in clinical trials to assess the impact of interventions on the patient's sense of well-being. The US Food and Drug Administration (FDA) has published the draft guidance for industry, "Patient-Reported Outcome Measures: Use in Medical Product Development to Support Labeling Claims," to inform sponsors, clinicians, and researchers of

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