Patient-Reported Outcome Measures in Systemic Sclerosis (Scleroderma)

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KEYWORDS

• Scleroderma • Systemic sclerosis • Patient-reported outcomes
• Health-related quality of life • Patient-reported outcome measures

KEY POINTS

- Systemic sclerosis (scleroderma) is a rare, chronic, connective tissue disease with fibrosis of the skin and many organs, vascular damage, and production of autoantibodies leading to many heterogeneous signs and symptoms.
- Systemic sclerosis is one of the most severe connective tissue diseases with disability; altered appearance; organ damage of skin, gastrointestinal tract, lungs, pulmonary arteries, kidneys, and other organs; digital ulcers; and amputation, with significant emotional and social impact.
- Patient-reported outcome measures provide a patient-centered method of assessing the impact of various problems in systemic sclerosis.
- Patient-reported outcome measures in systemic sclerosis can be general measures or tools that are unique to SSc.
- Commonly used patient-reported outcome measures include the Health Assessment Questionnaire Disability Index, Scleroderma Health Assessment Questionnaire, pain assessments, patient global assessments, Raynaud's Condition Score, and University of California, Los Angeles, Scleroderma Clinical Trials Consortium Gastrointestinal Scale 2.0.

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INTRODUCTION

Systemic sclerosis (SSc), also known as scleroderma, is a chronic, rare autoimmune disease involving the connective tissues of several organs in a progressive manner.[1] SSc classically is associated with fibrosis of the skin and internal organs, the production of autoantibodies, and vascular disruption and damage. Depending on the severity of the disease and the organs affected, patients with SSc have different clinical features.[2] Scleroderma is considered one of the most severe of the connective tissue diseases with significant morbidity and mortality.[3–5]

SSc is classified according to the amount of skin involvement. In limited cutaneous SSc, the skin distal to the elbows and knees is involved, but the skin of the face and neck may also be fibrosed. In the more severe diffuse cutaneous SSc (dcSSc) subset, skin fibrosis occurs both proximally and distally. The diffuse form is more progressive and has earlier visceral organ involvement.[6–9]

SSc usually causes fibrosis of the tissues of the face and hands, leading to disability and disfigurement.[10–12] However, many other manifestations of SSc can affect a patient's quality of life (QoL) and prognosis.[4,13,14] For example, SSc patients often experience pain from many features of their disease, including Raynaud's phenomenon, digital ulcers, gastrointestinal tract involvement, and inflammatory arthritis.[15,16] Moreover, SSc patients may experience severe pruritus, which can interfere with function and sleep.[17,18] Pulmonary involvement can include pulmonary arterial hypertension (PAH) and interstitial lung disease (ILD), resulting in symptoms such as dyspnea, fatigue, cough, and chest pain.[15,19] Chest pain in SSc may also be related to cardiac involvement and pleural and pericardial effusions. Gastrointestinal tract fibrosis can lead to dysmotility, severe gastroesophageal reflux disorder (GERD), early satiety, cramps, bloating, diarrhea and incontinence.[15,19] Scleroderma may affect the kidneys, leading to scleroderma renal crisis, or the heart, leading to cardiomyopathy, arrhythmias, and constrictive pericarditis.[19]

One of the most common symptoms in SSc is fatigue, which occurs in 90% of patients.[19] Other symptoms, including depressive symptoms, sexual dysfunction, fear of disease progression and death, issues with body image, and work disability all affect the QoL of patients with SSc.[19–25] Approximately half become work disabled.[12,23] Altered facial appearance and abnormal hands disfigure SSc patients, affecting self-esteem. Moreover, SSc patients suffer psychologically because of the progressive nature of SSc and lack of a cure.[24,25] There may be reduced oral opening and dry mouth, and combined with reflux this can result in problems eating, halitosis, and poor oral hygiene.

Treatment of SSc is generally complicated, including a combination of symptomatic treatment and disease- or organ-specific treatment. There are scant guidelines for nonpharmacologic management of SSc. Patient-reported outcome (PRO) measurements (PROMs) give patients the opportunity to express the effects of their disease experience. PROMs are unique in that they do not measure the same thing as biologic parameters and physician-assessed outcomes, even though there can be correlations between various measurements.[26]

Although objective measurements of disease characteristics related to SSc exist, such as pulmonary function tests or digital ulcer counts, which may overlap with PROS, they do not fully quantify the patient's experience. Physicians may view major internal organ involvement as extremely important, whereas patients may be more concerned with symptoms that reduce their QoL, such as intense pain, pruritus, or fatigue.[27] PROs allow for the measurement of health outcomes according to the patient, which reflect the goal of improving their QoL and health care. Most trials in SSc include outcomes reflecting laboratory, physician, and patient measures.