The critical need for patient-reported outcome measures to assess the severity and impact of systemic sclerosis

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Commentary

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Hand involvement is almost universal in patients with systemic sclerosis (SSc) and a major cause of pain and disability from the disease. The aetiology of hand involvement in SSc reflects the complexity of the disease and is often multi-factorial. This includes progressive skin fibrosis, joint contractures, musculoskeletal disease (e.g., inflammatory arthritis and myositis), vasculopathy e.g., (Raynaud's phenomenon and digital ulcers), and subcutaneous calcinosis[1-3]. There is also broad-ranging emotional impact including patients concerns about the physical appearance of their hands.

Patient-reported outcome measures (PROMs) provide valuable insights into the patient perspective of their disease. In SSc, PROMs are widely used in clinical practice and trials, including a number of SSc-specific instruments. However, a key issue for discernibility is that patients with SSc were largely not involved in the development of the majority of these instruments[4]. This is of key importance because PROMs should capture the multi-faceted impact and severity of disease and regulators require evidence of this to support drug labelling claims[5]. For example, the FDA require demonstration of clinical benefit (e.g., by feel, function and survival endpoints) for product approval.

In this issue of the *BJD*, Sibeoni et al[6]., report the development of a SSc PROM: the Hand scleroderma lived Experience (HAnDE) scale. The authors utilised a sequential mixed-method approach. The first phase was an inductive process to understand the lived experience of patients to generate a provisional 18-item scale. The second phase assessed the psychometric properties of the scale to validate the PROM, including reduction to 16-items. Internal consistency of the scale was excellent and construct validity was very good. Construct validity showed significant correlations with a number of widely used PROMs in SSc.

The HAnDE PROM was developed through a comprehensive approach including understanding the lived patient experience. However, there are a number of aspects to consider. The study was conducted in a single country (France) and patients were recruited from specialist centres, which could limit the generalisability of the PROM for patients with milder hand involvement not requiring speciality services. The authors highlight that

although the number of patients in phase two could appear small (n=105), no consensus exists on the minimum number required for principle-component analysis[6]. There were also differences in the patient characteristics between the two phases which could be important (e.g., presence of active digital ulcers and calcinosis).

The HAnDE is a welcomed PROM to assess the overall impact of hand involvement in SSc. The PROM captures the broad-ranging impact of SSc and mirrors recent qualitative work understanding the patient experience of SSc-digital vasculopathy[7-9]. This includes physical symptoms, impairment of physical and social activity, emotional impact including personal relationships, and impact of treatment. Of note, hand involvement has been reported to be a limitation to completion of PROMs in SSc[10]. Future research is warranted to further develop this comprehensive PROM for use in clinical practice and trials, including sensitivity to change to assess the impact of treatment interventions prescribed by rheumatologists and occupational therapists, and adaption for other languages.

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