

# Patient Preference Disability Questionnaire in Systemic Sclerosis: A Cross-Sectional Survey

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**Objective.** To assess patient priorities concerning disability in systemic sclerosis (SSc).

**Methods.** A total of 150 SSc patients (22 men) fulfilling the American College of Rheumatology and/or LeRoy and Medsger criteria for SSc were evaluated by the McMaster Toronto Arthritis Patient Preference Disability Questionnaire (MACTAR), Karnofsky performance status (KPS), Cochin Hand Function Scale, Health Assessment Questionnaire (HAQ), Hospital Anxiety and Depression Scale, Mouth Handicap in SSc (MHISS) scale, and global perception regarding their health status. Correlations between scores were analyzed using Spearman's coefficient. Logistic regression analysis was used to determine factors associated with patients' global perception of their health.

**Results.** Of the patients investigated, 81 (54%) had limited cutaneous SSc, 65 (43.3%) diffuse SSc, and 4 (2.7%) limited SSc. The 3 disability domains most often cited were walking (82 patients [54.6%]), housekeeping (67 patients [44.6%]), and sport activities (59 patients [39.3%]). The MACTAR score correlated moderately with KPS ( $r = 0.58$ ) but only weakly with the HAQ score ( $r = 0.38$ ). In multivariate analysis, 2 factors were associated with patients' negative global perception of their health status: KPS (odds ratio [OR] 1.07, 95% confidence interval [95% CI] 1.00–1.15) and MHISS score (OR 0.93, 95% CI 0.88–0.99).

**Conclusion.** For assessing SSc patient priorities concerning disability, the MACTAR has acceptable construct validity. Its weak correlation with the HAQ suggests that it adds useful information on disability.

## INTRODUCTION

Systemic sclerosis (SSc) is a connective tissue disease characterized by excessive collagen deposition, vascular

hyperreactivity, and obliterative microvascular phenomena (1,2). Patients with SSc are classified according to the extent of skin involvement: limited cutaneous SSc (lcSSc), with skin involvement essentially limited to the hands and face, visceral involvement usually limited to the digestive system and interstitium of the lung, and a good prognosis, with the exception of the 8–12% of patients in whom pulmonary arterial hypertension eventually develops (3,4); and diffuse SSc (dSSc), with proximal skin involvement and frequent visceral involvement, which is responsible for reduced life expectancy (5–7). In addition to diminishing life expectancy, SSc is responsible for skin, tendon, joint, and vessel damage, which leads to disability (8). Therefore, outcome measures with good metric properties that assess disability are needed to measure disease evolution and treatment efficacy in SSc.

Disability, the negative aspect of functioning, is now widely assessed in SSc, and several outcome measures of disability in SSc have been validated. Global disability is usually measured by the Health Assessment Questionnaire (HAQ) or the Scleroderma Health Assessment Questionnaire (SHAQ) (9). Recently, more detailed evaluation of disability has involved location-specific disability scales such as the Cochin Hand Function Scale (CHFS) (10,11) and the Mouth Handicap in Systemic Sclerosis (MHISS) scale (12). Thus, we have found that when assessed sepa-

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rately, the CHFS score contributes to 75% of the HAQ variance (11), whereas the MHSS score contributes to 36% of the HAQ variance (12), highlighting the need to specifically assess these disabilities in patients with SSc when evaluating treatment.

However, such measures of disability do not account for patient priorities. It has been shown that patients with rheumatoid arthritis (RA), health professionals, and healthy controls do not agree on the importance of disabilities (13). Taking into account such priorities may lead to a better understanding of what is important for patients and an increase in the validity and responsiveness of scales assessing disability (14). An example of a functional scale that investigates patient priorities is the McMaster Toronto Arthritis Patient Preference Disability Questionnaire (MACTAR) (15). The developers of the MACTAR noted good responsiveness for patients with RA in a controlled trial that revealed clinically important change, and its validity was reported in a multicenter randomized trial of RA (16). Using the MACTAR, an interviewer determines the activities patients are not able to perform that are the most important to the patients and probes whether this inability is due to the illness. Thus, the MACTAR concept of function may be more comprehensive than that of traditional fixed-item questionnaires and reveals issues that really matter to the patient.

To our knowledge, no study has investigated the application of the MACTAR in SSc. Therefore, we aimed to assess patient priorities in disability in a cohort of patients with SSc.

## PATIENTS AND METHODS

**Study design.** *Patients.* Patient members of the Association des Sclérodermiques de France (ASF), the French SSc patient association, were asked to participate in the survey during 3 consecutive annual meetings in 2004, 2005, and 2006. Eighty patients were asked to participate in 2004, and 50 (44 women) agreed; 98 patients were asked to participate in 2005, and 71 (59 women) agreed; and 95 patients were asked to participate in 2006, and 70 (55 women) agreed. To be eligible for the study, patients had to fulfill the American College of Rheumatology (formerly the American Rheumatism Association) (17) and/or the LeRoy and Medsger (18) criteria for SSc. Patients with localized scleroderma were excluded from the study. All patients were assessed by 1 of 4 operators (AB, FR, LM, and SP) during at least 1 of the 3 annual meetings in the spring (temperature 18–22°C).

Patients first had to complete self-questionnaires, then they underwent a 15-minute interview with a physician, allowing the physician to check for unanswered questions and complete questionnaires including clinical data.

**Demographic and clinical parameters.** Parameters recorded were age; sex; ethnicity; occupation; sick leave; year of onset of Raynaud's syndrome; age at diagnosis; year of onset of the first non-Raynaud's phenomenon; disease duration; disease form (limited SSc [lSSc], lcSSc, or dSSc); weight and size to calculate body mass index; interincisor

distance (measured in mm); dyspnea (assessed by the New York Heart Association 4-point scale); pitting scars; digital ulcers; calcinosis; esophagus, joint, and/or muscle involvement; heart involvement; interstitial lung disease; pulmonary arterial hypertension; and renal crisis. Evidence of esophagus, joint, and/or muscle involvement; heart involvement; interstitial lung disease; pulmonary arterial hypertension; and scleroderma renal crisis was based on patient reports. Physical ability was assessed using the Karnofsky performance status (KPS; 11 items ranging from normal health [100%] to death [0%], each scored as a percentage) (Appendix A). This scale is simple and easy to use.

In 2006, in addition to other assessments, patients were asked by interview whether their health status was perfectly acceptable, acceptable, poorly acceptable, or unacceptable using a 4-point semiquantitative scale, and esthetic burden was assessed by asking the patient the following question: "What is the global esthetic repercussion of your systemic sclerosis?" Responses were measured on an 11-point semiquantitative scale (range 0–10).

**Disability assessment.** Patients' priorities in disability were assessed using a French version of the MACTAR as described by Tugwell et al (15). Patients were first asked about activities affected by SSc. To assist the patient, the interviewer read a series of probing questions. The MACTAR questions are open ended and cover broad areas of function such as domestic care, self-care, professional activities, leisure activities, social interaction, and roles. Patients were encouraged to add activities not already listed. Then patients were asked to rank these activities in order of importance by answering the question, "Which of these activities would you most like to be able to do?" In a pilot study of 25 patients with SSc, RA, or generalized osteoarthritis, more than half of the French patients had difficulty identifying and ranking more than 3 items. Moreover, in the original report on the MACTAR, differences between analyses of 3-item and 5-item priority function were minimal (15). Thus, we used a 3-item priority function and asked patients to identify and rank 3 situations among activities of daily living that caused them maximal trouble. Items were not scored in the original MACTAR, but patients were asked if they had noticed changes in the problem they had identified several weeks earlier. In the validation study of the MACTAR, a Likert scale was added to quantify changes (16). In the present study, in order to reflect the degree of difficulty in performing a priority activity, each item was scored on an 11-point semiquantitative scale (range 0–10), with the global score ranging from 0 (no disability) to 30 (maximal disability).

Global disability was assessed using the HAQ (19), with the scale ranging from 0 (no disability) to 3 (maximal disability). The HAQ comprises 20 items divided into 8 domains and has been validated in French (20).

Hand disability was evaluated using the CHFS (21), which is administered by the physician, with 18 items concerning daily activities. Each question is scored on a scale of 0 (performed without difficulty) to 5 (impossible to

do). The total score is obtained by adding the scores of all items (range 0–90). This questionnaire has been validated in SSc (11).

Mouth disability was evaluated using the MHISS scale, which is administered by the physician, with 12 items concerning daily activities (12). Each question is scored on a scale of 0 (never) to 4 (always). The total score is obtained by adding the scores of all items (range 0–48). This questionnaire has been validated in French (12).

**Anxiety and depression assessments.** Anxiety and depression were assessed with the Hospital Anxiety and Depression Scale (HADS) anxiety and HADS depression (22). This scale includes 7 questions for anxiety and 7 for depression. Each point is scored 0–3 and the total score ranges from 0 (no depression, no anxiety) to 21 (maximal depression, maximal anxiety).

**Statistical analysis.** Data analysis was conducted using Systat 9 (SPSS, Chicago, IL). Quantitative variables are described as the mean  $\pm$  SD and range. Qualitative variables are described with proportions and percentages.

Correlations of the MACTAR score with other scores were assessed with the nonparametric Spearman rank coefficient ( $r$ ) because a normal distribution could not be demonstrated for all parameters studied. Spearman's correlation was interpreted as excellent ( $>0.91$ ), good (0.90–0.71), moderate (0.70–0.51), fair (0.50–0.31), or little or absent ( $<0.30$ ) (23). Nonparametric tests (Mann-Whitney) were used to compare the MACTAR total score according to type of SSc (lSSc or lcSSc versus dSSc) and acceptability (yes/no) of health status.

Stepwise logistic regression analysis was used to determine the variables associated with patients' global perception of their health (for this purpose, the variable was dichotomized as follows: perfectly acceptable/acceptable [yes] and poorly acceptable/nonacceptable [no]). Explanatory variables were introduced in the stepwise regression process if the difference in the scores was significant between patients who found their health status acceptable and those who found it not acceptable in univariate analysis. The maximum likelihood method of estimation was used. For all tests, the chosen level of significance was  $\alpha = 0.05$ .

## RESULTS

**Demographic and clinical data.** Overall, 150 patients were evaluated. Six patients were unable to define and rank disability priorities. All patients were white; 128 (85.3%) were female. The mean  $\pm$  SD age at the time of evaluation was 57.1  $\pm$  11.8 years and mean disease duration was 10.8  $\pm$  8.1 years.

Clinical parameters of patients are listed in Table 1. A total of 81 (54%) had lcSSc, 65 (43.3%) had dSSc, and 4 (2.7%) had lSSc. The mean KPS was 77  $\pm$  13 (range 60–100).

**Priority disabilities.** Priorities were individual and different for each patient. Overall, 37 different activities

**Table 1. Demographic and clinical characteristics of 150 SSc patients\***

Characteristic	Value
Age at the time of evaluation, years	57.1 $\pm$ 11.8
Female sex, no. (%)	128 (85.3)
Age at disease onset, years	46.2 $\pm$ 11.8
Disease duration at the time of evaluation, years	10.8 $\pm$ 8.1
Skin involvement, no. (%)	146 (97.3)
Limited SSc, no. (%)	4 (2.7)
Limited cutaneous SSc, no. (%)	81 (54)
Diffuse SSc, no. (%)	65 (43.3)
Raynaud's syndrome, no. (%)	147 (98)
Pitting scars, no. (%)	87 (58)
Digital ulcers, no. (%)	35 (23.3)
Calcinosis (n = 115), no. (%)	50 (43.5)
Gastrointestinal tract involvement, no. (%)	129 (86)
Arthralgias, no. (%)	117 (78)
Myalgias, no. (%)	108 (72)
Dyspnea (NYHA)	2.09 $\pm$ 0.74
Lung fibrosis, no. (%)	48 (32)
Pulmonary arterial hypertension, no. (%)	17 (11.3)
Renal crisis, no. (%)	7 (4.6)
Total MACTAR (range 0–30)	20.4 $\pm$ 7.1
Karnofsky performance status (range 0–100)	76.2 $\pm$ 12.7
Interincisor distance, mm	34.9 $\pm$ 7.4
Cochin Hand Function Scale (range 0–90)	21.1 $\pm$ 19.3
MHISS (range 0–48)	19.5 $\pm$ 10.4
HAQ (range 0–3)	1.2 $\pm$ 0.7
Anxiety (HADS) (range 0–21)	9.7 $\pm$ 4.4
Depression (HADS) (range 0–21)	7.0 $\pm$ 4.0
Esthetic burden (range 0–10)†	4.9 $\pm$ 2.5

\* Values are the mean  $\pm$  SD unless otherwise indicated. SSc = systemic sclerosis; NYHA = New York Heart Association; MACTAR = McMaster Toronto Arthritis Patient Preference Disability Questionnaire; MHISS = Mouth Handicap in Systemic Sclerosis scale; HAQ = Health Assessment Questionnaire; HADS = Hospital Anxiety and Depression Scale.

† n = 39 and n = 30 for limited cutaneous SSc and diffuse SSc, respectively.

were cited and ranked by the patients. Of these, 22 were cited less than 5 times and 12 were cited only once. The 10 activities that were more often cited were walking (54.6% of patients), housekeeping (44.6%), sports activities (39.3%), gardening (13.6%), shopping (11.2%), driving (10.5%), working (10.0%), cooking (9.3%), eating (8.6%), and social activities (8.0%). Twenty-two different activities were ranked as first priority; of these, the 3 priorities most often identified by patients as the first priority were walking (24.0%), housekeeping (23.3%), and sports activities (13.3%).

**Outcome measure scores.** The mean  $\pm$  SD MACTAR total score was 20.4  $\pm$  7.1. The mean HAQ global disability score was 1.2  $\pm$  0.7, the mean CHFS score was 21.1  $\pm$  19.3, and the mean MHISS score was 19.5  $\pm$  10.4. The observed mean scores of HADS anxiety and HADS depression were 9.7  $\pm$  4.4 and 7.0  $\pm$  4.0, respectively.

**Table 2. Correlation of the MACTAR score with other variable scores\***

Variable	MACTAR global score, Spearman's correlation coefficient (P)
Karnofsky performance status	0.58 (0.001)
Mouth opening	0.02 (0.72)
Cochin Hand Function Scale	0.21 (0.02)
MHISS	0.14 (0.29)
HAQ	0.38 (0.002)
Anxiety (HADS)	0.05 (0.73)
Depression (HADS)	0.37 (0.003)
Esthetic burden†	0.23 (0.08)

\* See Table 1 for definitions.  
 † Esthetic burden assessed on an 11-point semiquantitative scale (range 0–10).

**Correlation of the MACTAR score with other scores.** The MACTAR total score was best correlated with the KPS ( $r = 0.58$ ). Other correlations with the MACTAR score were global disability (HAQ;  $r = 0.38$ ), depression (HADS depression;  $r = 0.37$ ), esthetic burden (11-point semiquantitative scale;  $r = 0.23$ ), hand function (CHFS;  $r = 0.21$ ), anxiety (HADS anxiety;  $r = 0.05$ ), and mouth handicap (MHISS;  $r = 0.14$ ) (Table 2).

**Variables associated with patients' perception of their health status.** In univariate analysis, KPS, HAQ, MHISS, esthetic burden, and MACTAR total score differed significantly between patients who interpreted their health status as acceptable or not acceptable (Table 3). In stepwise logistic regression, we found that 2 variables were associated with patients' negative global perception of health status: KPS (odds ratio [OR] 1.07, 95% confidence interval [95% CI] 1.00–1.15) and MHISS score (OR 0.93, 95% CI 0.88–0.99).

**DISCUSSION**

Our results assessing patient priorities in SSc disability provide evidence that the MACTAR adds relevant infor-

mation when assessing patients with SSc. The weak correlation of the MACTAR with the HAQ and the large number of activities cited and ranked by patients suggest that the MACTAR adds useful information about patients' perceived disability. To our knowledge, this is the first assessment of the MACTAR scale for patients with SSc.

The MACTAR total score was only weakly correlated with the HAQ ( $r = 0.38$ ), which suggests that both instruments are not redundant and that disability priorities do not totally reflect global disability assessed with predetermined items. The weak correlation between the MACTAR and HAQ scores is not surprising considering that many of the 37 activities patients chose as priority disabilities are not represented among the daily activities assessed by the HAQ. For example, among the 3 most cited disability priorities, sports activities are not represented among the HAQ items. We assume that patients indicating sports activities as a priority disability participated in sports before their illness. The percentage of patients indicating sports activities as a first priority disability (13.3%) is the same as that of the general French population of the same age reporting participating in sports regularly. The mean HAQ score tended to be lower in patients indicating sports activities as the first priority disability than in the rest of the group (0.95 versus 1.28;  $P = 0.1$ ). It is difficult to speculate on whether the HAQ mean score was lower because patients participated in sports previously or because they wanted to participate in sports due to their better health.

The SHAQ might have been more tightly correlated with the MACTAR. Steen and Medsger proposed the SHAQ for measuring SSc status and changes in disease status (9). They demonstrated that visual analog scale scores for digital ulcers, gastrointestinal symptoms, and lung symptoms correlated with findings for these organ systems and their changes over time (9). However, we (11) and others (20) failed to demonstrate differences between both questionnaires in lcSSc and dSSc, probably because Raynaud's phenomenon, digital ulcers, gastrointestinal symptoms, and lung symptoms occur with both subsets of the disease. Finally, we observed a high correlation between the HAQ

**Table 3. MACTAR and other variable scores according to type of SSc and patient opinion on acceptability of health status\***

Score	Type of SSc			Patient opinion of health status		
	ISSc or lcSSc (n = 85)	dSSc (n = 65)	P†	Acceptable (n = 41)	Not acceptable (n = 28)	P†
Total MACTAR (range 0–30)	19.9 ± 5.8	21.1 ± 8.3	0.39	19.0 ± 4.9	22.6 ± 5.5	0.01
Karnofsky performance status (range 0–100)	76.9 ± 14.6	75.2 ± 9.7	0.40	80.5 ± 9.2	74.3 ± 8.4	0.005
Interincisor distance, mm	35.7 ± 7.4	33.8 ± 7.3	0.18	35.1 ± 6.7	34.1 ± 7.3	0.60
Cochin Hand Function Scale (range 0–90)	17.9 ± 17.8	25.2 ± 20.5	0.02	17.5 ± 17.9	24.7 ± 18.7	0.12
MHISS (range 0–48)	17.7 ± 10.1	21.8 ± 10.5	0.04	16.8 ± 10.0	23.1 ± 8.9	0.008
HAQ (range 0–3)	1.1 ± 0.7	1.4 ± 0.7	0.07	1.0 ± 0.7	1.4 ± 0.7	0.03
Anxiety (HADS) (range 0–21)	9.2 ± 4.4	10.2 ± 4.5	0.21	10.0 ± 4.3	10.2 ± 4.6	0.27
Depression (HADS) (range 0–21)	6.8 ± 4.0	7.3 ± 4.0	0.51	6.1 ± 3.3	7.7 ± 3.8	0.08
Esthetic burden (range 0–10)‡	4.6 ± 2.5	5.2 ± 2.5	0.39	4.3 ± 2.6	5.6 ± 2.1	0.03

\* Values are the mean ± SD unless otherwise indicated. SSc = systemic sclerosis; ISSc = limited SSc; lcSSc = limited cutaneous SSc; dSSc = diffuse SSc; see Table 1 for additional definitions.  
 † Comparison between ISSc or lcSSc and dSSc and between patient opinion status acceptable versus not acceptable. Comparisons were performed using Mann-Whitney test.  
 ‡ n = 39 for ISSc and lcSSc and n = 30 for dSSc.

and aggregate SHAQ scores ( $r = 0.88$ ), which suggests that the information added by the SHAQ may not be essential when assessing global disability in patients with SSc (11).

The MACTAR and CHFS scores were only weakly correlated, probably due to the same reasons as the weak correlation between the MACTAR and HAQ scores. Among the 3 most cited disability priorities, hand ability is required only for housekeeping activities. Although not negligible, mouth disability was not often chosen as a disability priority by SSc patients. Eating was the ninth disability priority cited, and among other disability priorities involving the mouth, laughing and drinking sparkling water were cited once. This observation probably also explains the low correlation between the MHISS and MACTAR scores.

Interestingly, the MACTAR score was correlated fairly strongly with the KPS ( $r = 0.58$ ). The KPS was developed in cancer patients, and is a strong predictor of cancer outcome (24,25). Thus, use of the KPS offers a clinical estimate of a patient's physical state, performance, and prognosis after a therapeutic procedure and a patient's suitability for therapy. Although we agree that SSc is not cancer, the KPS appeared helpful to predict outcome in many acute or chronic diseases, as different as stroke (26), chronic diarrhea in immunocompromised patients (27), or chronic graft-versus-host disease (28). Moreover, the KPS may be used among other parameters to study the outcome of patients with chronic fatigue or disability as the main symptom (29). Therefore, we do consider SSc a severe disease and some patients with SSc may have a low KPS, which is why we chose it for this study.

Finally, an important observation is that MACTAR scores were significantly higher in patients who found their health status to be unacceptable than in those who found it acceptable. This strengthens the assumption that recording priority disabilities is relevant because patients' global opinion of their health status can be considered as an external standard.

One limitation of our study may be the procedure used to recruit patients. Because all patients belonged to the French association of patients, they may not be representative of the entire French SSc population. Therefore, HAQ scores for patient members of this association are quite high and comparable with those reported in a previous study conducted in a tertiary care setting (20). In addition, patients had longstanding disease, which could imply more symptoms. Therefore, further evaluation in other cohorts of patients with SSc is necessary to confirm the construct validity of the MACTAR in SSc.

The MACTAR needs to be administered by trained interviewers and might not be an easy, cost-effective instrument to use routinely (16). This point should be assessed in further studies.

Finally, we did not assess sensitivity to change of the MACTAR. For RA, changes in disease status and patients' adaptation to their limitations have been shown to shift patient priorities, therefore long-term followup with the MACTAR may be questionable (16). Moreover, it is not clear whether the MACTAR would measure change rather than simply unrealistic desires. These aspects should be

assessed in SSc before the scale is used to evaluate treatment efficacy.

In conclusion, the MACTAR scale, taking into account patient priorities of disability, is highly associated with patient opinion of health status and weakly associated with other disability scores in SSc. This score can add nonredundant and pertinent information to results from other scales for assessing the health status of patients with SSc. The MACTAR might be considered as an outcome measure in future trials of scleroderma after its sensitivity to change is ascertained.

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## AUTHOR CONTRIBUTIONS

Dr. Mouthon had full access to all of the data in the study and takes responsibility for the integrity of the data and the accuracy of the data analysis.

**Study design.** Mouthon, Rannou, Fermanian, Poiraudou.

**Acquisition of data.** Mouthon, Rannou, Bérezné, Guilpain, Goldwasser, Guillevin, Poiraudou.

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**Manuscript preparation.** Mouthon, Rannou, Bérezné, Pagnoux, Goldwasser, Guillevin, Poiraudou.

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#### APPENDIX A: KARNOFSKY PERFORMANCE STATUS

100	Able to work. Normal; no complaints; no evidence of disease.
90	Able to work. Able to carry on normal activity; minor symptoms.
80	Able to work. Normal activity with effort; some symptoms.
70	Independent; not able to work. Cares for self; unable to carry on normal activity.
60	Disabled; dependent. Requires occasional assistance; cares for most needs.
50	Moderately disabled; dependent. Requires considerable assistance and frequent care.
40	Severely disabled; dependent. Requires special care and assistance.
30	Severely disabled. Hospitalized, death not imminent.
20	Very sick. Active supportive treatment needed.
10	Moribund. Fatal processes are rapidly progressing.