Does Incorporation of Aids and Devices Make a Difference in the Score of the Health Assessment Questionnaire–Disability Index? Analysis from a Scleroderma Clinical Trial

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ABSTRACT. ***Objective.*** The Health Assessment Questionnaire-Disability Index (HAQ-DI) is a commonly used musculoskeletal-targeted measure in systemic sclerosis (SSc). We assessed if HAQ-DI scores are different when calculated with and without aids/devices, and if apparent responsiveness changes when scored in these 2 ways.

Methods. We used data from a placebo-controlled clinical trial in diffuse SSc. Baseline HAQ-DI total score was calculated with and without aids/devices and compared using Student's t-test. We also classified the HAQ-DI scores into no-to-mild disability (0.00–1.00), moderate disability (1.01–2.00), and severe disability (2.01–3.00). Responsiveness to change was evaluated using the effect size (ES).

Results. The mean (SD) baseline HAQ-DI score was 1.33 (0.68) with aids/devices compared to HAQ-DI score 1.16 (0.70) without aids/devices ($p = 0.03$). When the baseline HAQ-DI score was categorized into no-to-mild, moderate, and severe disability, the proportion of patients in the no-to-mild disability (29% with aids/devices vs 44% without aids/devices) and moderate disability (59% with aids/devices vs 45% without aids/devices) groups were statistically different ($p < 0.001$). The ES was similar between the 2 groups (ES = 0.01 and 0.02 with and without aids/devices).

Conclusion. This analysis suggests a shift from no-to-mild disability to moderate disability when aids/devices are incorporated in total HAQ-DI score. Future clinical trials in SSc should explicitly state whether HAQ-DI score was calculated using aids/devices. (First Release Dec 15 2007; J Rheumatol 2008;35:466–8)

Key Indexing Terms:
HEALTH ASSESSMENT QUESTIONNAIRE–DISABILITY INDEX
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The Health Assessment Questionnaire-Disability Index (HAQ-DI)[1] is the most commonly used musculoskeletal-targeted measure of functional ability in systemic sclerosis (SSc)[2]. The HAQ-DI is a 20-item self-administered questionnaire that is scored from 0 (no disability) to 3 (severe disability). The HAQ-DI is reliable and responsive to change in SSc[3,4], meets the OMERACT criteria[2], and predicts morbidity and mortality in SSc[5]. Each item in the HAQ-DI has a companion aids/devices variable that is used to record what type(s) of assistance, if any, the participant uses for his/her usual activities. It is recommended that the scoring is adjusted by including the use of these aids/devices[1,6]. However, in SSc, published data on HAQ-DI from clinical trials and longitudinal studies either did not use aids/devices[3,5,7,8] or do not provide information whether aids/devices are included or not[9-11].

We used data from a randomized, placebo-controlled, NAIMS/NIAID-sponsored clinical trial involving 168 patients with diffuse SSc randomized to receive either 12 months of daily oral bovine collagen therapy or placebo[12,13] to assess if HAQ-DI scores are different when calculated with and without aids/devices, and if responsiveness to change differed

among the 2 groups. This study was chosen as it included HAQ-DI along with aids/devices items. There was no statistically significant difference in the change in the skin score at Month 12 in the 2 groups in the trial as a whole, so we grouped patients from both study arms together for this analysis.

Statistical analysis. The baseline total HAQ-DI score was calculated with and without aids/devices and compared using Student's t-test. The adjustment of total HAQ-DI score was performed as recommended by Fries[14]. If aids/devices and/or help from another person are checked for a category, the score is set to 2, unless the score is already 2. If the score is 3, it remains a 3. In other words, scores of 0 or 1 are increased to 2. For example, if the highest score for the "arising" category is 1, and the patient states they use a device for arising, the computed category score would be 2. The sum of the computed categories scores is then calculated and divided by the number of categories answered. This gives a score in the range of 0 to 3.

We also classified the baseline HAQ-DI scores into no-to-mild disability (score 0.00–1.00), moderate disability (1.01–2.00), and severe functional disability (2.01–3.00)[15,16] and compared the differences using the chi-square test. Responsiveness to change was evaluated using the effect size (ES). For ES, the mean change in the HAQ-DI from the baseline to Month 12 is divided by the standard deviation at baseline. All analysis was performed using Stata software, version 9.2 (Stata, College Station, TX, USA). P values < 0.05 were considered to indicate statistical significance.

RESULTS

Baseline characteristics. The mean (SD) age of participants was 50.8 (12.2) years and all participants had early [disease duration = 3.5 (2.7) years] diffuse SSc. The mean baseline HAQ-DI score was 1.33 (0.68) with aids/devices compared to HAQ-DI score of 1.16 (0.70) without aids/devices ($p = 0.03$). The proportions of patients in the no-to-mild disability (29% with aids/devices vs 44% without aids/devices) and moderate disability groups (59% with aids/devices vs 45% without aids/devices) were statistically different ($p < 0.001$; Figure 1). The proportion of patients in the severe disability category was similar between the 2 groups — 12% versus 11% calculated with and without aids/devices ($p > 0.05$). ES was similar between the 2 groups (ES = 0.01 with aids/devices and 0.02 without aids/devices).

DISCUSSION

Functional disability is an important outcome measure in scleroderma (SSc) clinical trials and the HAQ-DI has been incorporated in virtually all clinical trials conducted recently[3,9,10,17]. One advantage of uniform incorporation of HAQ-DI is potential comparison of disease severity across SSc groups and across musculoskeletal disorders.

In this population with early diffuse SSc, the mean baseline HAQ-DI score was statistically different: 1.33 (SD 0.68) with aids/devices compared to 1.16 (SD 0.70) without aids/devices ($p = 0.03$). In a recent analysis of another clinical trial of early diffuse SSc, a difference of 0.14 in HAQ-DI was clinically meaningful[18]. Therefore, the difference in HAQ-DI seen with and without aids/devices of 0.17 in the current analysis is both statistically significant and clinically meaningful. In addition, when the HAQ-DI score was grouped into no-to-mild, moderate, and severe functional disability, higher proportions of scores with aids/devices were categorized into moderate disability (59%) compared to higher proportion that were classified as no-to-mild disability (44%) when aids/devices were excluded. In other words, the subjects who were classified as having borderline moderate disability when aids/devices were included were reclassified as mild disability when aids/devices were excluded, suggesting a distribution shift. This shift shows the effect of using different procedures for determining the HAQ-DI and demonstrates the importance of using a uniform procedure for determining the HAQ-DI score. On the other hand, proportions of subjects with severe disability were similar with (12%) and without (11%) aids/devices since most of the subjects with severe disability are unable to do activities of daily living. This finding has notable implications for classification of patients with mild, moderate, and severe disability for clinical and health services research. Our data suggest that categorization of patients into mild and moderate disability in diffuse SSc for research purposes will significantly alter the results of the analysis.

Our other objective was to assess if using aids/devices affects responsiveness to change in a clinical trial; inclusion of aids/devices did not influence the direction and magnitude of change in this population. In other words, inclusion of aids/devices in SSc clinical trials may not affect the responsiveness to change, an important item in determining the validity of an instrument, and this shows that the direction and degree of change is not highly dependent on the specific procedure for doing the HAQ-DI.

The limitations of this analysis are that it is based on a single study in diffuse SSc and did not include any patients with limited SSc. However, most recent clinical trials in SSc were performed in patients with early diffuse SSc. Second, although HAQ-DI has been shown to discriminate between active treatment and placebo in rheumatoid arthritis clinical trials[19], this is not possible in this analysis as there was no difference between placebo and collagen groups at 1 year. However, HAQ-DI is responsive to change in relationship to disease severity in early diffuse SSc[3].

Our data suggest that calculation of HAQ-DI total score with and without aids/devices leads to a statistically significant and clinically meaningful difference in the apparent disability of patients with SSc. These results should be confirmed in another SSc clinical trial. Future clinical trials in SSc should explicitly state whether HAQ-DI scores were calculated using aids/devices. The categorization of patients into groups of mild, moderate, and severe disability has implications for clinical and health services research.

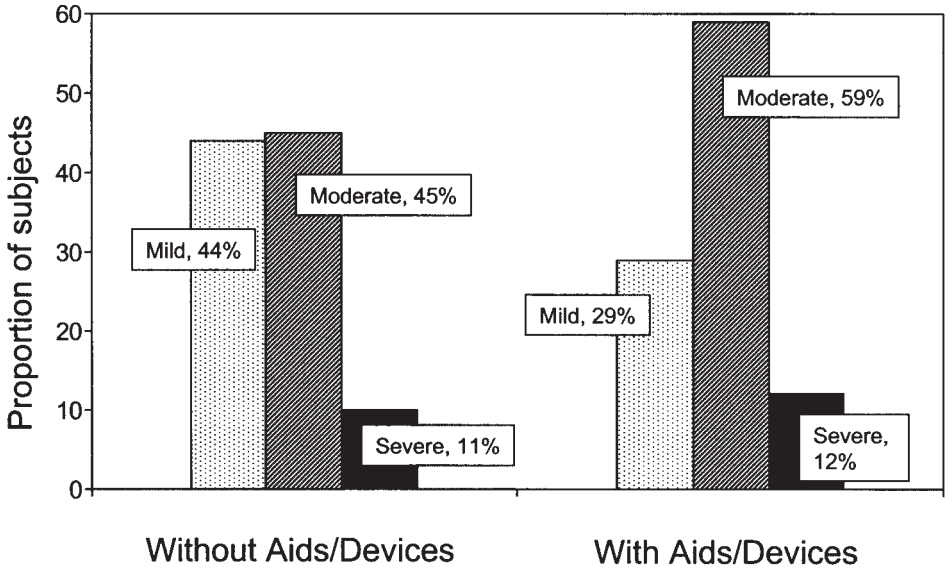

Figure 1. Proportion of subjects who are classified into groups of no-to-mild, moderate, and severe functional disability when the Health Assessment Questionnaire-Disability Index score is calculated with and without aids/devices.

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REFERENCES

1. Fries JF, Spitz P, Kraines RG, Holman HR. Measurement of patient outcome in arthritis. Arthritis Rheum 1980;23:137-45.
2. Furst D, Khanna D, Matucci-Cerinic M, et al. Systemic sclerosis — continuing progress in developing clinical measures of response. J Rheumatol 2007;34:1194-200.
3. Khanna D, Furst DE, Clements PJ, et al. Responsiveness of the SF-36 and the Health Assessment Questionnaire Disability Index in a systemic sclerosis clinical trial. J Rheumatol 2005;32:832-40.
4. Khanna D, Merkel PA. Outcome measures in systemic sclerosis: an update on instruments and current research. Curr Rheumatol Rep 2007;9:151-7.
5. Clements PJ, Wong WK, Hurwitz EL, et al. The Disability Index of the Health Assessment Questionnaire is a predictor and correlate of outcome in the high-dose versus low-dose penicillamine in systemic sclerosis trial. Arthritis Rheum 2001;44:653-61.
6. Bruce B, Fries JF. The Stanford Health Assessment Questionnaire: a review of its history, issues, progress, and documentation. J Rheumatol 2003;30:167-78.
7. Cole JC, Khanna D, Clements PJ, et al. Single-factor scoring validation for the Health Assessment Questionnaire-Disability Index (HAQ-DI) in patients with systemic sclerosis and comparison with early rheumatoid arthritis patients. Qual Life Res 2006;15:1383-94.
8. Khanna D, Clements PJ, Furst DE, et al. Correlation of the degree of dyspnea with health-related quality of life, functional abilities, and diffusing capacity for carbon monoxide in patients with systemic sclerosis and active alveolitis: results from the Scleroderma Lung Study. Arthritis Rheum 2005;52:592-600.
9. Merkel PA, Herlyn K, Martin RW, et al. Measuring disease activity and functional status in patients with scleroderma and Raynaud's phenomenon. Arthritis Rheum 2002;46:2410-20.
10. Denton CP, Merkel PA, Furst DE, et al. Recombinant human anti-transforming growth factor beta-1 antibody therapy in systemic sclerosis: A multicenter, randomized, placebo-controlled phase I/II trial of CAT-192. Arthritis Rheum 2006;56:323-33.
11. Pope JE, Bellamy N, Seibold JR, et al. A randomized, controlled trial of methotrexate versus placebo in early diffuse scleroderma. Arthritis Rheum 2001;44:1351-8.
12. Postlethwaite A, Furst D, Wong WK, Clements P. Oral tolerance (OT) induction to type I collagen (CI) significantly reduces the skin score in patients with diffuse systemic sclerosis (SSc) with late-phase disease. Results of a NIAMS/NIAID multicenter phase II placebo-controlled double blind clinical trial [abstract]. Arthritis Rheum 2005.
13. Khanna D, Furst DE, Wong WK, et al. Reliability, validity, and minimally important differences of the SF-6D in systemic sclerosis. Qual Life Res 2007;16:1083-92.
14. The Health Assessment Questionnaire. [Internet. Accessed November 21, 2007.] Available from: http://www.chcr.brown.edu/pcoc/EHAQDESCRSCORINGHAQ372.pdf
15. Krishnan E, Sokka T, Hakkinen A, Hubert H, Hannonen P. Normative values for the Health Assessment Questionnaire disability index: benchmarking disability in the general population. Arthritis Rheum 2004;50:953-60.
16. Krishnan E, Tugwell P, Fries JF. Percentile benchmarks in patients with rheumatoid arthritis: Health Assessment Questionnaire as a quality indicator (QI). Arthritis Res Ther 2004;6:R505-R513.
17. Khanna D, Yan X, Tashkin DP, et al. Impact of oral cyclophosphamide on health-related quality of life in patients with active scleroderma lung disease: Results from the scleroderma lung study. Arthritis Rheum 2007;56:1676-84.
18. Khanna D, Furst DE, Hays RD, et al. Minimally important difference in diffuse systemic sclerosis — results from the D-Penicillamine Study. Ann Rheum Dis 2006;65:1325-9.
19. Strand V, Cohen S, Crawford B, Smolen JS, Scott DL. Patient-reported outcomes better discriminate active treatment from placebo in randomized controlled trials in rheumatoid arthritis. Rheumatology Oxford 2004;43:640-7.