ABSTRACT NUMBER: 1079

The Potential Effect on Recruitment of Restricting Skin Scores Eligibility Criteria in Early Diffuse Scleroderma Trials

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Meeting: 2015 ACR/ARHP Annual Meeting

Date of first publication: September 29, 2015

Keywords: clinical trials, scleroderma and systemic sclerosis

SESSION INFORMATION

Date: Sunday, November 8, 2015

Session Title: Systemic Sclerosis, Fibrosing Syndromes, and Raynaud's - Clinical Aspects and Therapeutics I

Session Type: ACR Concurrent Abstract Session

Session Time: 4:30PM-6:00PM

Background/Purpose: There is increasing interest in cohort enrichment for clinical trials of early diffuse SSc (dcSSc). Recent EUSTAR database analysis (Maurer et al. 2015) suggests that patients with a low modified Rodnan skin score (mRss)< 22 and short disease duration (< 15 months from first non-Raynaud symptom) have the highest probability of worsening (defined as progressive skin involvement) at one year. The autoantibody profile in the US dcSSc population is different than Europe, with greater percentages of patients with anti-RNA polymerase III (RNAP) antibody in the US. Anti-RNAP positive patients often present with higher skin scores. If an upper threshold skin score is used as an inclusion criterion thenmany RNAP positive patients could be excluded. The objective of this study was to examine the effect of restricting mRss in early diffuse SSc clinical trials with respect to: 1)the percentage of patients experiencing a significant change in mRss and 2) recruitment in a US population.

Methods: We used a single-center cohort of prospectively followed early dcSSc patients seen for an initial visit between Jan. 1, 1980 and Dec. 31, 2013 at a US Scleroderma Center. Early was defined as < 18 months from the first non-Raynaud symptom. Patients had to have at least two mRss within

one year of the first SScCenter visit. After descriptive baseline statistics, time to peak skin score, and the percentage of patients who had improvement or worsening of mRss over one year were calculated. Data is presented by skin score at presentation.

Results: Among 304 eligible patients, mean age at the first SSc Center visit was 51.8 ±13.5 years. The cohort was 76% female and 93%Caucasian. Overall patients were 58% RNAP positive, 21% anti-Scl70 positive, 11% other SSc-autoantibody positive and 10% unknown. The percentage of patients with improving or worsening mRss by ≥5 points,≥5 points and 25% change, and the times to peak skin score are shown in Table 1. Including patients with higher baseline skin score did not change the overall % of patients improving or worsening their mRss within one year. The median time to peak skin score was nearly identical in the patient groups. By restricting our inclusion criterion to mRss< 25 points, 27-40% of patients with worsening mRss would have been excluded with different progression.

	Table 1: Skin score change at one year by baseline mRss at presentation				
mRss at first SSc Center visit	**N**	**mRssworsened by ≥5 points**	**mRss improved by ≥5 points (%)**	**< 5 point change in mRss**	**Median (IQR) time to peak mRss from first visit**
10-25	176	87 (49%)	47 (27%)	42 (24%)	0.45 (0.00, 0.64)
10-30	217	110 (51%)	55 (25%)	52 (24%)	0.46 (0.16,0.67)
10-35	253	127 (50%)	69 (27%)	57 (23%)	0.45 (0.15, 0.66)
10-40	284	142 (50%)	82 (29%)	60 (21%)	0.42 (0.07, 0.66)
10-45	304	143 (47%)	90 (30%)	71 (23%)	0.40 (0.00, 0.63)
	N	**mRssworse ≥5 points and 25%**	**mRss improved by ≥5 points and 25%**	**< 5 point and 25% change in mRss**	
10-25	176	84 (47%)	44 (25%)	58 (33%)	
10-30	217	97 (45%)	49 (22%)	71 (33%)	

10-35	253	108 (43%)	61 (24%)	84 (33%)	
10-40	284	115 (41%)	69 (24%)	100 (35%)	
10-45	304	115 (38%)	71 (23%)	118 (36%)	

Conclusion: In one
US SSc Center population expanding the allowable mRss from ≥22 to ≤45 did not decrease the percentof
patients changing their mRss. Restricting mRss at≤
22 may significantly limit our potential to recruit patients in the US. Further study of this issue should be
undertaken with additional modeling and consideration to different autoantibody frequencies in geographic regions.
Limitations of our data include that it is single center
population.

References: Maurer et
al., Annals Rheum Dis. 2015; 74: 1124.

Disclosure: R. T. Domsic, Biogen-Idec, 5,Bayer, 5; **D. Khanna**, Bristol-Myers Squibb, 2,EMD Serono, 2,Genentech and Biogen IDEC Inc., 2,Bayer, 5,Biogen Idec, 5,Cytori, 5,EMD Serono, 5,Forward, 5,Genentech and Biogen IDEC Inc., 5,Gilead, 5,Lycera, 5,Seattle Genetics, 5; **M. Lucas**, None; **V. D. Steen**, None; **D. E. Furst**, Gilead, 2,GlaxoSmithKline, 2,NIH, 2,Novartis Pharmaceutical Corporation, 2,Pfizer Inc, 2,Roche Pharmaceuticals, 2,Genentech and Biogen IDEC Inc., 2,UCB, 2,Abbvie, 5,Actelion Pharmaceuticals US, 5,Amgen, 5,Bristol-Myers Squibb, 5,Cytori, 5,Janssen Pharmaceutica Product, L.P., 5,Gilead, 5,GlaxoSmithKline, 5,NIH, 5,Novartis Pharmaceutical Corporation, 5,Pfizer Inc, 5,Roche Pharmaceuticals, 5,Genentech and Biogen IDEC Inc., 5,UCB, 5,Abbvie, 8,Actelion Pharmaceuticals US, 8,Bristol-Myers Squibb, 2,Amgen, 2,Actelion Pharmaceuticals US, 2,Abbvie, 2,UCB, 8; **R. Lafyatis**, Shire, Sanofi, Regeneron, Genentech, UCB, HGS, Precision Dermatology, Biogen, BMS, Inception, Stromedix, PRISM, Pfizer, 2,Shire, Sanofi, Regeneron, Roche/Genentech, Biogen, Lycera, Novartis, Celgene, BMS, Amira, Celdara, Celltex, Dart Therapeutics, Idera, Inception, Intermune, Medimmune, Precision Dermatology, Promedior, Zwitter, PRISM, UCB, Actelion, EMD Sereno, Akros, E, 5; **T. A. Medsger Jr.**, None.

To cite this abstract in AMA style:

Domsic RT, Khanna D, Lucas M, Steen VD, Furst DE, Lafyatis R, Medsger TA Jr.. The Potential Effect on Recruitment of Restricting Skin Scores Eligibility Criteria in Early Diffuse Scleroderma Trials [abstract]. *Arthritis Rheumatol.* 2015; 67 (suppl 10). http://acrabstracts.org/abstract/the-potential-effect-on-recruitment-of-restricting-skin-scores-eligibility-criteria-in-early-diffuse-scleroderma-trials/. Accessed October 2, 2015.

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