Safety and Efficacy of Subcutaneous Tocilizumab in Systemic Sclerosis

This study explored the safety and efficacy of tocilizumab for the treatment of diffuse cutaneous sclerosis in systemic sclerosis and specifically reported the results of an open label extension trial.

The tocilizumab study looked at patients who had diffuse cutaneous disease and they were relatively earlier in their disease course. The patients were either randomized to tocilizumab versus placebo for 48 weeks, and the primary endpoint for this study was the change in their skin score, which we also call MRSS or Modified Rodnan Skin Score.

What they found in this study was, in fact, there was no advantage to treating patients with tocilizumab in terms of their change in skin score. But in the study, they did find that the patients who had been randomized to tocilizumab had less of a decline in their forced vital capacity or lung function compared to those patients who had been randomized to placebo.

The present study is reporting the results of the open label extension trial. This is when everyone in the trial gets the opportunity to go on treatment with tocilizumab, including those patients who had been randomized to placebo early on. What they found is that even though those patients who had been randomized to placebo had experienced a loss of lung function early on, once they got put on the tocilizumab in the open label extension trial, they had stabilization of their lung function. Similarly, those patients who had been in the tocilizumab arm from the beginning, they remained on tocilizumab and continued to have sustained stability in their lung function. So I think that this result is very important in terms of reassuring us that tocilizumab may play a disease-modifying role in slowing the progression of interstitial lung disease in patients with systemic sclerosis. But future studies are needed to really look at patients who only have interstitial lung disease.

This study looked at all patients with systemic sclerosis and diffuse disease, both with and without ILD. So an important next step would be you do a study but just have patients with ILD, to truly see whether again tocilizumab can improve lung function in patients with SSc-ILD. So the treatment of SSc-ILD can be quite complex, and it can be based on patient's underlying disease features, how established they are in their disease course. I would consider using tocilizumab in a patient who is similar to a patient who participated in this trial. So namely, a patient who has early disease, has diffuse cutaneous disease, and also some evidence of systemic inflammation, so in this study the patients had to have elevated levels of inflammatory markers in their blood. Based on this study, this might be the ideal patient population to try tocilizumab in for the treatment of SSc-ILD.

Currently with systemic sclerosis in patients who have tight skin and pulmonary changes, they're often given mycophenolate and sometimes cyclophosphamide. In this particular study, tocilizumab statistics were not obtained, but it appears to have some at least modest effects on skin loosening and improvements in forced vital capacity. It should be noted that skin scores do improve naturally with time and after a year or two in patients with systemic sclerosis, so we really couldn't say much about the

comparisons other than initial studies are needed in order to ascertain whether subcutaneous tocilizumab should be used in patients with systemic sclerosis with skin or pulmonary involvement.