AB0649

B CELL DEPLETION THERAPY IN VERY EARLY SYSTEMIC SCLEROSIS. A POTENTIAL WINDOW OF OPPORTUNITY?

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Background: Our group, among others, has shown that B cell depletion therapy may improve skin thickening and interstitial lung disease (ILD) related to systemic sclerosis (SSc). All studies assessing the clinical efficacy of B cell depletion therapy in SSc have recruited patients with established disease fulfilling the old classification criteria. In our previous studies we have found that patients with shorter disease duration had a better clinical response to RTX.

Objectives: It is unknown whether treatment in very early SSc can affect long term outcomes. We aimed at assessing the effect of rituximab (RTX) in patients with very early SSc.

Methods: We report 2 cases where RTX was administered within 24 months from the appearance of Raynaud's.

Results: A 56 year-old female with an 18-month history of Raynaud's developed interstitial lung disease (ILD) with extensive ground glass lesions, a normal FVC (82%) but decreased DLco (44%). A diagnosis of SSc was made based on the presence of positive anti-centromere antibodies, puffy fingers, telangiectasias, positive capillaroscopy, ILD and Raynaud's. Six months following RTX treatment, her PFT's had increased (FVC 94% and DLco 55%) and chest HRCT showed an obvious improvement. One year later the patient no longer reported dyspnea. She remained on RTX for 5 years with no respiratory symptoms, stable PFTs and no evidence of disease progression. The second case is a 27-year old female with a 1-year history of Raynaud's, puffy fingers, positive anti-Scl70 and capillaroscopy. Within a few months her skin thickened rapidly reaching an MRSS of 12 and developed tendon friction rubs. Six months following RTX treatment skin thickening had almost resolved; she only had mild sclerodactyly. Tendon friction rubs disappeared. The patient received 8 consecutive RTX courses throughout a period of 4 years. During this period the patient remained in a steady clinical condition with no signs of disease progression.

Conclusion: It is generally accepted that autoimmunity/immune dysregulation and vasculopathy are the primary events in SSc that eventually trigger the fibrotic process. Based on this pathogenetic model, one can hypothesize that a therapeutic intervention very early in the disease course, prior to the appearance of fibrotic manifestations could potentially prevent permanent organ damage. Our data suggest that there may be a window of opportunity in SSc. This is why we propose that large scale, controlled studies assessing the efficacy of RTX (or other immune based therapies such as mycophenolate) in patients with very early disease are highly needed.

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PERSPECTIVES AND UNMET NEEDS OF PATIENTS WITH POOR PROGNOSIS SYSTEMIC SCLEROSIS ON AUTOLOGOUS HEMATOPOIETIC STEM CELL TRANSPLANTATION CARE: A QUALITATIVE STUDY

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Background: Autologous hematopoietic stem cell transplantation (HSCT) can induce long-term remission in patients with diffuse cutaneous systemic sclerosis (dcSSc). Nevertheless, it is an intensive treatment with risk of treatment-related mortality and associated adverse events requiring long-term hospital admission. Little is known about the psychosocial impact of HSCT on patients with dcSSc. Additionally, post-discharge care is currently not standardized.

Objectives: To gain more insight in the experiences of patients during and after HSCT and to identify unmet needs in current care.

Methods: Semi-structured interviews were conducted with dcSSc patients that underwent HSCT in four academic hospitals in the Netherlands. Interviews were transcribed verbatim and analyzed with NVIVO12.

Results: Eight patients were interviewed (5 males and 3 females, mean age 48 years (SD 10), mean disease duration 4.6 years (SD 1.9), mean time since HSCT 3.2 years (SD 2.4). At inclusion mean SHAQ was 1.3 (SD 1.1) and mean EQ5D-5L 0.86 (SD 0.21). While patients were satisfied with the frequent, low-threshold contacts with physicians and nurses, the decision-making process followed by HSCT and recovery was mentally taxing. Although patient education was experienced as extensive pre-treatment, participants reported they only felt the real impact of it during therapy. Patients felt anxious throughout the hospital stay and they could not recall most events from that period. Feelings of losing control over health and over time were frequently described. Support of family and friends was important during treatment. Apart from the practical aid, they provided emotional support, helping to cope with emotions and to find purpose. Furthermore, a specialized nurse involved in psychosocial support and care management was highly valued. Still, participants felt lonely and misunderstood due to invisible disabilities, such as fatigue and pain. After HSCT, patients experienced more physical limitations than they expected, i.e. low energy levels and limited hand function. They worried about contracting infections and relapse risk. Professional rehabilitation was often hindered. There were unmet needs. Patients preferred to be better informed about what to expect after admission. Structured and written instructions should include themes such as self-management, practical tips and prognosis of recovery. Participants would advise peers to keep a diary and even take photos during HSCT and hospital stay, in order to help recollect and assimilate past events. Peer-support was recommended by most patients.

Conclusion: HSCT had a large impact, both physical and emotional. We identified unmet needs regarding patient education on rehabilitation and psychosocial support in the period following HSCT.

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THE INFLUENCE OF RITUXIMAB THERAPY ON THE DYNAMICS OF THE BASIC PARAMETERS AND ACTIVITY OF SYSTEMIC SCLEROSIS

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Background: Systemic sclerosis (SSc) is an autoimmune disease characterized by skin and multi-organ involvements induced by overproduction of autoantibodies by B cells.

Objectives: To assess the influence of Rituximab (RTX) therapy on the dynamics of the basic parameters and activity of SSc during long-term follow-up.

Methods: There were 90 SSc patients (pts) in the prospective study. 75% were women. 57% pts had diffuse SSc. SSc was confirmed ACR/EULAR criteria. The average duration of the disease was 5.8 years and of the study was 27 months. For the purpose of the analysis, a group of pts was selected that had no less than two evaluation points from 12 to 42 months and having received 2.9±1.1 g RTX in average during such period. The examination included both routine clinical laboratory parameters and special research. We evaluated the modified Rodnan skin score (mRSS), forced vital capacity (FVC), diffusing capacity of carbon monoxide (DLCO), the Valentini Disease Activity index, estimated glomerular filtration rate (eGFR) before and after completed RTX. All pts included in the study received treatment with vascular, anti-inflammatory and/or immunosuppressive medicines. The average dose of prednisolone was 11.8±4.4 mg/day.

Results: In general, more than 90% pts demonstrated different kinds of improvements. A reliable improvement was documented based on a number of indicators, which reflect typical features of the disease. Thus, skin induration was reduced (skin score decreased), lung functional capacity