Pediatric Rheumatology

Poster presentation

Successful autologous stem cell transplantation (ASCT) in a patient with juvenile dermatomyositis

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Juvenile dermatomyositis (JDM) is a chronic inflammatory disorder, which primarily affects muscle and skin. Patients usually present with progressive muscle weakness accompanied by an erythematous rash over the joints and across the face. Articular, cardiac, pulmonary, and gastrointestinal manifestations may occur resulting in severe morbidity.

We report a 16 year old patient, who was diagnosed with JDM four years ago with severe muscle weakness and skin involvement. Despite therapy including methotrexate, steroids, immunoglobulins, cyclosporine A and rituximab a sustained remission could not be achieved. The patient developed progressive contractures due to persistent inflammatory reactions. Due to treatment-refractory disease immunoablation followed by an autologous stem cell transplantation (ASCT) was performed. The stem cells were mobilized by application of 2 g/m^2 cyclophosphamide. After immunoablation using ATG (thymoglobuline®) 10 mg/kg, cyclophosphamide 120 mg/kg and fludarabine 150 mg/m^2, CD3/CD19-depleted CD34+stem cells (7.5 × 10^6/kg) including 2,9 × 10^4/kg T-cells were transferred. The haematological reconstitution with leucocytes >1000/ml and granulocytes >500/µl was achieved on day +7 and day +8, respectively. Substitution of erythrocytes or platelets was not necessary. No severe infections or organ toxicity (WHO) were observed.

During the 9 months after this therapy a marked improvement of the patient was observed clinically and in the MRI. The Childhood Myositis Assessment Scale (CMAS) changed from 6 to 43, the Karnofsky index increased from 50% to 90% post ASCT.

Taken together we could demonstrate for the first time that an ASCT is a therapeutic option with low toxicity for patients with severe, therapy-refractory JDM.