AB0925

TOCILIZUMAB AS A TREATMENT OPTION FOR PATIENTS WITH JUVENILE SYSTEMIC SCLEROSIS

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Background: Juvenile systemic sclerosis (JSS) is a rare autoimmune disease characterized by skin stiffness and fibrosis of internal organs (eg. lung, heart, gastrointestinal system). The efficacy and safety of tocilizumab (TCZ), an interleukin 6 receptor- α inhibitor, in adult patients have been studied in a few recent studies. Reports on its efficacy among children have been reported among patients with localized disease form. This is a first report on treatment of JSS patients with TCZ.

Objectives: We aimed to evaluate efficacy of tocilizumab as a treatment option for patients with juvenile systemic sclerosis. Consequently, we sought to explore its influences on internal organ involvement, skin stiffness and patient global assessment score.

Methods: We retrospectively evaluated patients diagnosed with JSS according to EULAR criteria. Among 30 patients diagnosed with JSS, 7 (23%) were treated with TCZ. Demographic data are taken from patients' charts. Data on clinical characteristics and treatment outcome are recorded during the patients' last visit.

Results: Seven patients treated with TCZ were analyzed: 6 (85.71%) girls, one boy. Median age of patients was 17.5 years (range:11-12 years), at disease onset 10 years (range 6-15 years) and at diagnosis 11 years (range 7.5-17 years). Median disease duration was 8 years (min. 3-max.11 years). Median duration of TCZ treatment was 10 months (min. 1-max. 21 months). One (14.28%) patient had gastro-intestinal involvement, 4 (57.14%) patients had lung involvement and 2 (28.57%) patients had both gastro-intestinal and lung involvement. Improvement in mRSS was detected in 5 (71.43%) patients. DLCO improved in 4 (57.14%) patients. Four (57.14%) patients had radiologically confirmed improvement in lung findings. Data on radiological findings of one patient treated with TCZ for 6 months were not available. Five patients (71.43%) reported improvement in patient global assessment (PGA) score after the TCZ treatment. PGA score of two remaining patients were not applicable due to short treatment duration (Table 1). None of 7 patients had recorded adverse effect of TCZ treatment.

Abstract AB0925 Table 1. Clinic characteristics of juvenile systemic sclerosis patients patients treated with tocilizumab

Lung involvement	GIS involvement	Treatment duration (months)	TCZ treatment duration (months)	Radiological improvement in thorax HRCT	Pre-/Post-treatment DLCO (%)	Pre-/Post-treatment mRSS	Pre-treatment PGA
+	+	78	12	+	52/52	21/19	5
-	+	90	6	NA	93/95	33/22	2
+	+	108	21	+	65/71	32/22	4
+	-	96	11	+	54/62	28/16	3
+	-	66	10	+	107/125	28/24	4
+	-	30	1	NA	51/NA	12/NA	3
+	-	12	1	NA	NA/NA	32/NA	5

Conclusion: JSS is rare condition characterized with internal organ involvement. Tocilizumab represents an efficacy treatment options for patients unresponsive to standard treatment. Long-term prospective studies with higher number of patients are needed to provide more relevant data.

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JUVENILE SYSTEMIC SCLEROSIS AND MUCINOUS ADENOCARCINOMA OF THE LUNG IN PATIENT WITH CYSTIC ADENOID MALFORMATION-PARANEOPLASTIC SYNDROME OR JUST A COINCIDENCE?

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Background: Juvenile systemic sclerosis (JSS) is a rare auto-immune condition characterized by the skin stiffness and internal organ fibrosis. In adult patients, systemic sclerosis has been related to an increased risk of malignancy with lung cancer being most prevalent. Up-to-date, we haven't found any report on lung cancer in patient with JSS.

Objectives: Herein, we aimed to present a case with juvenile systemic sclerosis and coincidentally detected cystic adenoid malformation complicated by the mucinous adenocarcinoma of the lung.

Methods: Data on disease history are taken from patient's chart. Clinical and laboratory findings are recorded during the last visit at our outpatient department.

Results: A 14 years old previously healthy female patient, admitted to our outpatient department due to puffy fingers, Raynaud's phenomenon and stiffness of the skin proximally to metacarpophalangeal joints. Her anti-nuclear antibody was positive (1/80) but the other auto-antibodies (including Anti Scl 70, anti-centromere antibody) were negative. Patient was diagnosed as progressive systemic sclerosis and the prednisolone, methotrexate and nifedipine were started. Routinely performed echocardiography was normal. Respiratory function tests were appropriate for the age and hemoglobin level: FVC 80%, DLCO 92%. Surprisingly, thorax HRCT revealed cystic adenoid malformation on the left lower lobe of the lung so pediatric surgery department has been consulted. The decision for surgical intervention has been made and the lesion has been removed. Tissue sample has been sent to the pathological investigation and the result was consistent with mucinous adenocarcinoma of the lung. There were 2 focus (0.4 cm in diameter) of the adenocarcinoma in the cystic adenoid malformation without invasion to the local perineural, lymphatic or vascular tissues, with positive KRAS (KRAS12-612C, 612S) mutation. Cranial MRI and PET-CT show no residual, relapse nor the metastatic lesions. Clinical follow-up has been suggested, without need for further oncological treatment.

Figure 1. Thorax HRCT of patient

Conclusion: Juvenile systemic sclerosis is a rare disease characterized with multiorgan involvement. The paraneoplastic syndrome with clinical presentation of systemic sclerosis should be kept on mind among adolescents, especially those with negative autoantibodies.

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