Abstract #:20

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JAK Inhibitors: a Promising Molecular-Targeted Therapy in Dermatomyositis.

Objectives: We previously observed in vitro that IFN-I reproduces dermatomyositis (DM) pathological findings, that pathogenic effects may be prevented in vitro by JAK inhibitor (JAKinh) therapy and an improvement was observed clinically using JAKinh in four refractory DM patients. Our objective was to expand this observation clinically and to describe the evolution of refractory DM patients treated with JAKinh.

Methods: DM patients were considered refractory if the disease remained active after at least two different lines of immunosuppressive therapy combined with corticosteroids (CS) +/- intravenous immunoglobulins (IVIg). Disease activity was assessed using the Medical Research Council scale (MRC-5) and the Cutaneous DM Disease Area and Severity Index (CDASI) every 3 months. Health Assessment Questionnaire (HAQ) and modified Rankin Scale (mRS) were performed to assess patients reported quality-of-life and disability, respectively. Tolerability and adverse events were also monitored.

Results: Seven refractory DM patients treated with JAKinh were identified. All patients were females, mean age at diagnosis was 49.5±25.6 years and most patients (n=6/7) presented a myositis-specific autoantibody (TIF1gamma n=5; SAE n=1). One patient presented an ovarian cancer at initial diagnosis, 7 years prior to JAKinh therapy. On average, these patients previously received 3±1 line of immunosuppressive therapy and all of them IVIg. At JAKinh initiation (ruxolitinib n=5; baricitinib n=2), mean disease duration was 8.7±12.8 years, CS dose was 6±4 mg/day, all other immunosuppressive agents were discontinued and 2 patients were treated with IV Ig concomitantly. At baseline, mean CDASI-activity score was 38±10, deltoid MRC-5 was 4.4±0.6, psoas MRC-5 was 4.1±0.8, CK level was 439±936, HAQ was 1.2±0.5 and mRS was 1.0±0.9. At 3-months follow-up, a significant improvement of CDASI-activity score (>5 points) was observed in all patients, mean CDASI-activity score was 20±11, deltoid MRC-5 was 4.7±0.4, psoas MRC-5 was 4.4±0.4, CK level was 158±90, HAQ was 0.8±0.4 and mRS was 0.5±0.5. Mean CS dose was 6±4 mg/day and 2 patients had reduced their monthly IV Ig dose. At last-follow up, mean treatment duration with JAKinh was 11.2±4.0 months and all patients were still receiving JAKinh therapy. Over this period, 1 patient presented a herpes zoster infection. Another was briefly hospitalized for a non-severe pneumonia and a superficial thrombophlebitis, and presented one year later a deep vein thrombosis and pulmonary embolism requiring hospitalization.

Conclusions: Altogether this case series support the use of JAKinh in the management of refractory cutaneous DM patients demonstrating a sustained remission at one year.