An open label study of tofacitinib in the treatment of vitiligo

Alopecia areata (AA) is a autoimmune disease of melanocytes affecting 1%–2% of the population. It can occur at any age but is most common before the age of 20 years.
Current treatments for severe disease are limited and there is significant unmet patient need for safe and effective treatments for vitiligo.
Gene association studies identified the JAK/STAT pathway to be involved in the pathogenesis of vitiligo.
Tofacitinib is a novel selective JAK 3 Janus kinase inhibitor. Small case series and phase 1 Clinical Trials indicate that tofacitinib might also be effective in the treatment of vitiligo.
In order to investigate to potential role of tofacitinib in the treatment of vitiligo, we enrolled 25 patients with vitiligo refractory to conventional treatment in an open label pilot study of both oral and/or topical tofacitinib. Initial dose was 2.5 mg once daily. This was titrated up or down according to patient response and tolerability.
The median age was 50 years (14–72) and there were 9 females and 16 males.
Patients were stratified according to disease severity which was assessed by body surface area of depigmentation.
The primary end point was the percentage change in pigmentation during treatment. No pertinent discontinued the medication due to side effects. Our study further supports the use of tofacitinib in this population.