RISING DERM STARS

Topical JAK inhibitor Ruxolitinib for Vitiligo Treatment

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Background: The often visible, disfiguring lesions of vitiligo may have a major impact on patient quality of life. Unfortunately, existing therapies for vitiligo are limited in efficacy and can be associated with undesirable side effects. Janus kinase (JAK) inhibitors such as ruxolitinib may offer a new and safe therapeutic option for vitiligo treatment by targeting the T-helper 1 (Th1) cell immune mediated pathway that defines vitiligo pathogenesis.

Methods: A 20-week open-label proof-of-concept trial of twice daily topical application of ruxolitinib 1.5% cream in 12 adult patients with a minimum of 1% affected body surface area (BSA) of vitiligo. Topical application was limited to 10% BSA to avoid systemic side effects. The primary outcome was improvement in Vitiligo Assessment Severity Index (VASI) from Baseline to week 20.

Results: Of the 12 patients screened, 11 patients enrolled and 9 patients completed the study (mean age 51.72, male 54%). A 23% (95% CI: 4% - 43%, p=0.02) improvement in overall VASI scoring from Baseline to week 20 was observed. An approximately 76% (95% CI: 53% - 99%, p=0.001) improvement in facial VASI scoring was observed in four patients with significant facial involvement at Baseline (Figure 1, Figure 2). Three patients experienced minimal non-statistically significant re-pigmentation in vitiligo located on the body and 1 patient had slight acral re-pigmentation (Figure 1). Adverse events were minor including erythema, hyperpigmentation and transient acne on skin where ruxolitinib was applied. As laboratory monitoring was not performed in our patients after the Baseline visit, the authors cannot comment on potential laboratory adverse events, but it was assumed these were not likely to occur with topical application.

Conclusion: Twice daily application of topical ruxolitinib 1.5% cream provided significant facial vitiligo re-pigmentation and may offer a valuable new treatment for vitiligo. After the conclusion of this study, the trial was extended 32 weeks in 8 patients, 4 of whom experienced an additional statistically significant improvement in facial VASI [92% ± 7.1%, (P = .0001)] at 52 weeks. Three of 6 patients experienced re-pigmentation of non-acral upper extremity vitiligo (12.6% ± 19.5%), two of whom underwent simultaneous nbUVB phototherapy and had previously failed phototherapy and topical ruxolitinib 1.5% cream as single agent treatment regimens. A multi-centered phase 2 randomized controlled trial was designed based on the encouraging results of our proof-of-concept study and is currently underway at 26 research sites throughout the country.

Figure 1: Percent Improvement in VASI Scoring.

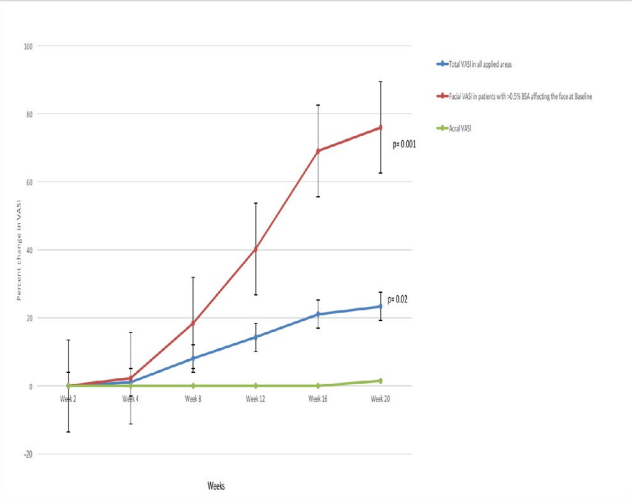
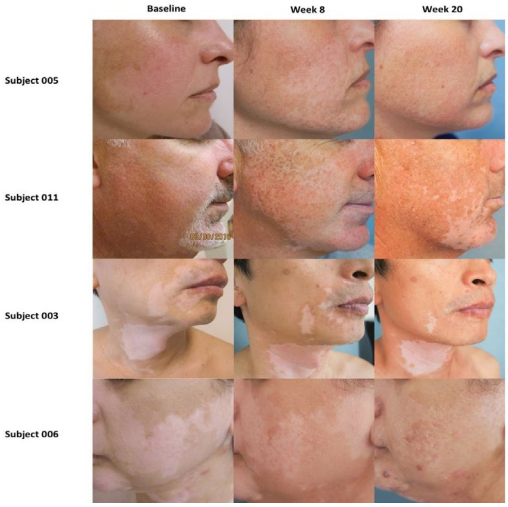


Figure 2: Clinical Improvement in Facial Vitiligo.



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