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Efficacy of ruxolitinib cream in vitiligo by patient characteristics and affected body areas: Descriptive subgroup analyses from a phase 2, randomized, double-blind trial

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Letter

Research letter

Efficacy of ruxolitinib cream in vitiligo by patient characteristics and affected body areas: Descriptive subgroup analyses from a phase 2, randomized, double-blind trial

To the Editor: Vitiligo is a chronic autoimmune disease resulting in patches of depigmented skin[1] and reduced quality of life.[2] In a randomized, dose-ranging phase 2 study (NCT03099304) in 157 adult patients, the Janus kinase (JAK) 1/JAK2 inhibitor ruxolitinib cream produced substantial repigmentation of facial and total body vitiligo lesions after 24 weeks, with continued improvement through week 52, and was well tolerated.[3] Here, we present treatment response subanalyses from the phase 2 trial.

The proportion of patients receiving 1.5% ruxolitinib cream twice daily who achieved ≥50% improvement in facial Vitiligo Area Scoring Index (F-VASI50) at week 24 was assessed by demographics and baseline clinical characteristics. Additionally, the proportion of patients initially randomized to 1.5% ruxolitinib cream once daily or twice daily who achieved ≥50% and ≥75% improvement from baseline in total VASI (T-VASI50 and T-VASI75, respectively) at week 52 was assessed by affected body area. Because the ruxolitinib cream application was limited to ≤20% of total body surface area (T-BSA; the limit for the practicality of application), total body analyses were conducted only in patients with vitiligo affecting ≤20% of T-BSA at baseline. Data were analyzed using descriptive statistics.

Among the 33 patients who received 1.5% ruxolitinib cream twice daily, a larger proportion of F-VASI50 responders at 24 weeks were aged ≤50 years compared with >50 years (58.8% vs 31.3%; Fig 1). A larger proportion of women versus men (60.0% vs 33.3%) were F-VASI50 responders. A larger proportion of responders had ≤1.5% affected baseline facial BSA, disease duration >20 years, and previous treatment with phototherapy. There were no substantial differences between responders based on race, skin type, baseline T-BSA, or disease status.

Among patients with vitiligo affecting ≤20% of T-BSA at baseline, both doses of ruxolitinib cream (1.5% once daily and twice daily) produced notable T-VASI50 and T-VASI75 responses at week 52 (Table I). The 1.5% ruxolitinib cream twice-daily dose produced the highest proportion of T-VASI50 responders in the head/neck region (60.0%), followed by the upper and lower extremities (52.9% and 52.6%, respectively). T-VASI50 of the hands and feet was noted for 15.0% and 29.4% of patients, respectively, who received 1.5% ruxolitinib cream twice daily.

In summary, ruxolitinib cream demonstrated trends for clinical activity for treating vitiligo across demographics and clinical characteristics, including in patients with longstanding and extensive disease. F-VASI50 responses were observed in >40% of patients previously treated with topical corticosteroids or topical calcineurin inhibitors and in two-thirds of patients who received prior phototherapy. Ruxolitinib cream also produced a clinically meaningful repigmentation of all body areas, including acral areas, which are notoriously difficult to repigment.[4]

Overall, these findings support the use of ruxolitinib cream for treating vitiligo. Reported analyses were descriptive, and sample sizes were small, warranting confirmation of results in larger populations.

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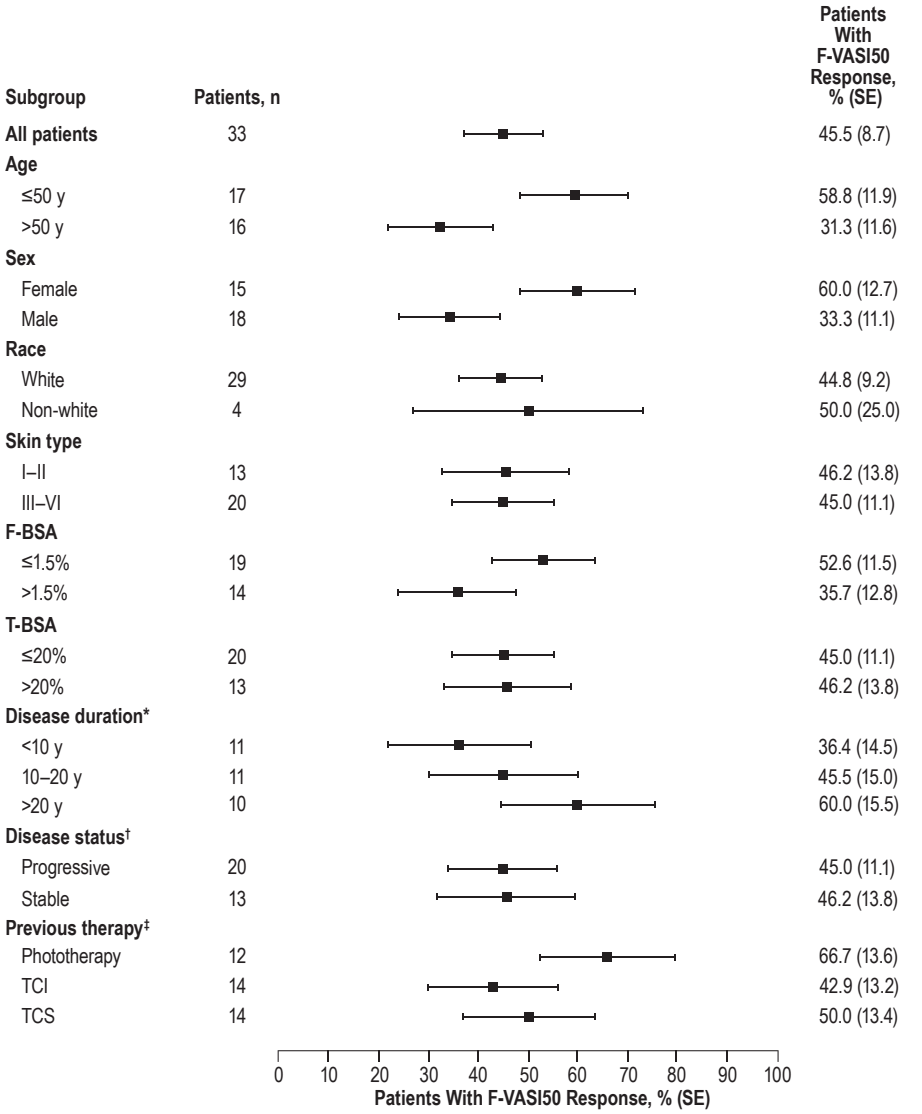

Fig 1. F-VASI50 response to 1.5% ruxolitinib cream twice daily at week 24 by patient demographics and clinical characteristics. *Disease duration was not available for 1 patient; † Determination of disease stability was based on investigator judgment. ‡ Patients could have used ≥1 previous therapy. *F-BSA*, Facial body surface area; *F-VASI50*, ≥50% improvement from baseline in facial Vitiligo Area Scoring Index; *SE*, standard error; *TCI*, topical calcineurin inhibitor; *TCS*, topical corticosteroid; *T-BSA*, total body surface area.

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Conflicts of interest

Dr Hamzavi has served as an advisory board member for AbbVie; a consultant for Incyte Corporation, Pfizer, and UCB; a principal investigator for AbbVie, Allergan, Bayer, Clinuvel Pharmaceuticals, Estée Lauder, Ferndale Laboratories, Galderma Laboratories LP, GE Healthcare, Incyte Corporation, Janssen, Janssen Biotech, Johnson & Johnson, Lenicura, LEO Pharma, Pfizer, and Unigen; a subinvestigator for Amgen, Bristol Myers Squibb, Foamix Pharmaceuticals, and Janssen; president of the HS Foundation; and cochair of the Global Vitiligo Foundation. Dr Rosmarin has received honoraria as a consultant for AbbVie, Celgene, Dermavant Sciences, Dermira, Eli Lilly and Company, Janssen, Kyowa Kirin,

Table I. Proportion of patients who applied 1.5% ruxolitinib cream and achieved T-VASI50 or T-VASI75 responses at week 52*

	Ruxolitinib cream	
Responders, n/N (%)	**1.5% once daily**	**1.5% twice daily**
T-VASI50†	7/19 (36.8)	9/20 (45.0)
Head/neck	6/19 (31.6)	12/20 (60.0)
Trunk	7/18 (38.9)	5/17 (29.4)
Upper extremities	7/18 (38.9)	9/17 (52.9)
Lower extremities	6/18 (33.3)	10/19 (52.6)
Hands	4/19 (21.1)	3/20 (15.0)
Feet	5/19 (26.3)	5/17 (29.4)
T-VASI75†	2/19 (10.5)	3/20 (15.0)
Head/neck	5/19 (26.3)	11/20 (55.0)
Trunk	4/18 (22.2)	2/17 (11.8)
Upper extremities	3/18 (16.7)	4/17 (23.5)
Lower extremities	3/18 (16.7)	5/19 (26.3)
Hands	4/19 (21.1)	1/20 (5.0)
Feet	4/19 (21.1)	3/17 (17.6)

T-BSA, Total body surface area; *T-VASI50*, ≥50% improvement from baseline in total Vitiligo Area Scoring Index; *T-VASI75*, ≥75% improvement from baseline in total Vitiligo Area Scoring Index.
*T-VASI50 and T-VASI75 responses are reported for the subset of patients with baseline T-BSA ≤20% because treatment was limited to lesions constituting ≤20% of T-BSA.
†Percentage change from baseline differs for each body region, and the value for all body regions is based on the sum of all regions; binary outcomes (ie, yes/no) for T-VASI50 may not follow the distribution of percentage change from baseline.

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