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Total Vitiligo Area Scoring Index Response Maintenance or Shift During 52 Weeks of Ruxolitinib Cream Treatment for Vitiligo: Pooled Analysis of the TRuE-V Phase 3 Studies

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Introduction

- Vitiligo is a chronic autoimmune disease that targets melanocytes, resulting in patches of skin depigmentation[1]
- Disease pathogenesis is largely regulated by interferon-γ activation of the Janus kinase (JAK) signaling pathway[2]
- A cream formulation of ruxolitinib, a JAK1/JAK2 inhibitor, was recently approved by the US Food and Drug Administration for the topical treatment of nonsegmental vitiligo in adult and pediatric patients 12 years of age and older[3]
- In 2 randomized, double-blind, vehicle-controlled phase 3 studies of adults and adolescents with vitiligo (TRuE-V1 [NCT04052425]/TRuE-V2 [NCT04057573]), ruxolitinib cream was statistically superior to vehicle at Week 24 in the primary and all key secondary efficacy endpoints[4]

Objective

- To evaluate shifts (improvement or decrease) or stability in total Vitiligo Area Scoring Index (T-VASI) response among patients who applied ruxolitinib cream for 52 weeks in TRuE-V1/TRuE-V2

Methods

Patients and Study Design

- For both studies, eligible patients were aged ≥12 years with a diagnosis of nonsegmental vitiligo and depigmented areas covering ≤10% total body surface area (BSA; facial and nonfacial), including ≥0.5% BSA on the face and ≥3% BSA on nonfacial areas, with scores ≥0.5 on facial VASI (F-VASI) and ≥3 on T-VASI
- Key exclusion criteria and study design (**Figure 1**) were described previously[4]

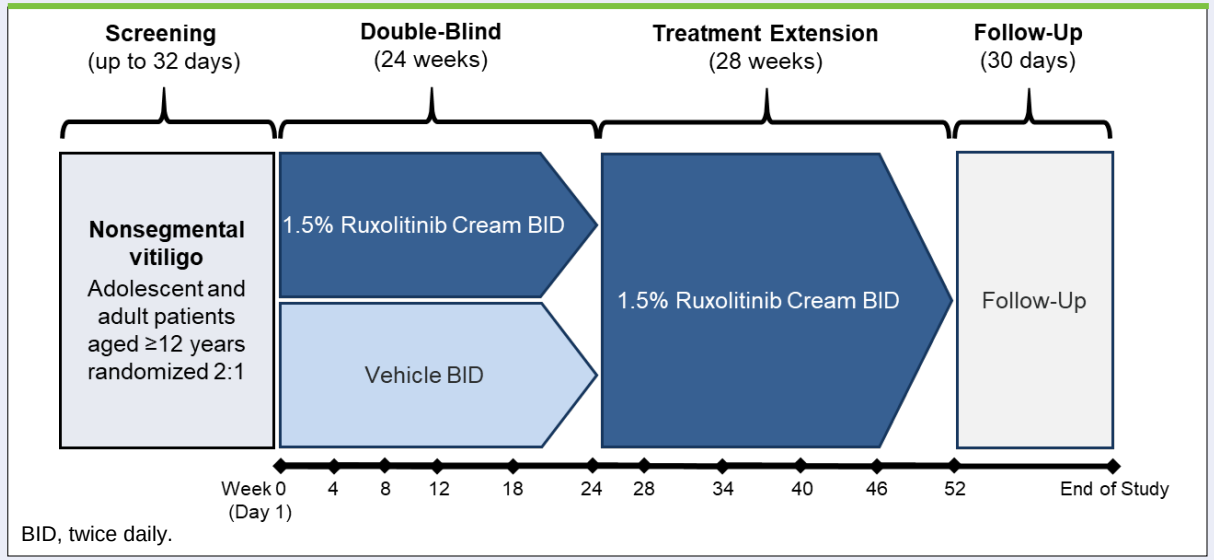
Endpoints and Assessments

- T-VASI responses were examined (eg, T-VASI50 represents ≥50% improvement from baseline) among patients who applied ruxolitinib cream and had nonmissing evaluations at Week 12
- The safety and tolerability of ruxolitinib cream were also assessed

Statistical Analyses

- Efficacy data at Weeks 12, 24, and 52 are reported as observed, and subgroup analyses were summarized using descriptive statistics
- All patients who applied ≥1 dose of study drug were included in the safety analysis

Figure 1. Study Design



BID, twice daily.

Results

Patients

- In total, 450 patients were randomized to apply ruxolitinib cream in the TRuE-V studies; 449 patients were included in the safety analysis
- In the ruxolitinib cream group, mean (SD) age at baseline was 39.4 (15.4) years, 44.8% of patients were male, 71.5% had Fitzpatrick skin types I–III, and median (range) duration of vitiligo was 11.9 (0–60.5) years
- Baseline mean (SD) F-VASI and T-VASI scores were 0.92 (0.55) and 6.67 (2.05), respectively

T-VASI Response Shifts or Stability

- In total, 401 patients who applied ruxolitinib cream from Day 1 had T-VASI evaluations at Week 12, 10.7% of whom achieved T-VASI50 or better (**Table 1; Figure 2**)
  - At Week 24, 34.4% of patients improved their response from Week 12
  - Over half (58.4%) remained stable in their Week 12 response
  - 3.5% decreased their response
  - 3.7% had missing evaluations

Table 1. Response Shift From Week 12 to Week 24 of Treatment

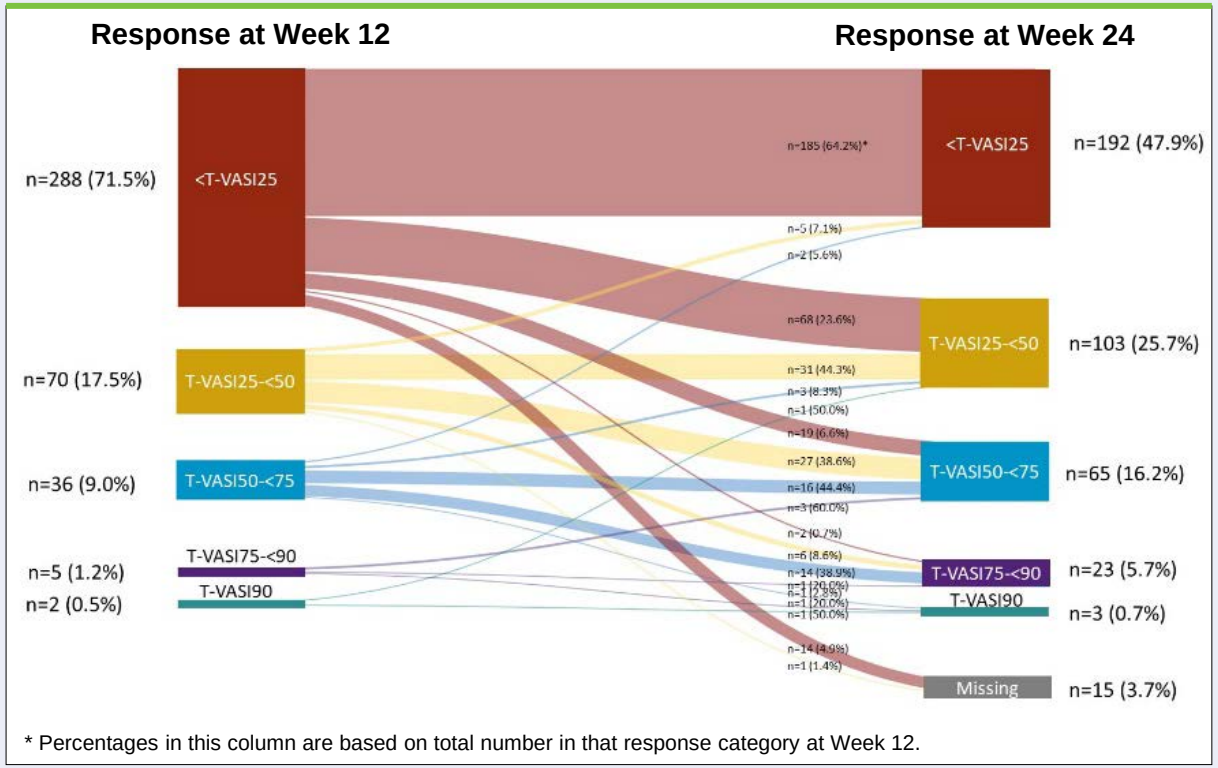
Response	Response at Week 12, n (%)*	Response at Week 24, n (%)† <T-VASI25	T-VASI 25–<50	T-VASI 50–<75	T-VASI 75–<90	T-VASI ≥90	Missing
<T-VASI25	288 (71.8)	185 (64.2)	68 (23.6)	19 (6.6)	2 (0.7)	0	14 (4.9)
T-VASI25–<50	70 (17.5)	5 (7.1)	31 (44.3)	27 (38.6)	6 (8.6)	0	1 (1.4)
T-VASI50–<75	36 (9.0)	2 (5.6)	3 (8.3)	16 (44.4)	14 (38.9)	1 (2.8)	0
T-VASI75–<90	5 (1.2)	0	0	3 (60.0)	1 (20.0)	1 (20.0)	0
T-VASI ≥90	2 (0.5)	0	1 (50.0)	0	0	1 (50.0)	0
Total	401 (100)	103 (47.3)	103 (25.7)	65 (16.2)	23 (5.7)	3 (0.7)	15 (3.7)

T-VASI, total Vitiligo Area Scoring Index; T-VASI25, ≥25% improvement from baseline; T-VASI50, ≥50% improvement from baseline; T-VASI75, ≥75% improvement from baseline; T-VASI90, ≥90% improvement from baseline.

* Percentage based on total evaluable patients (n=401).

† Percentage based on total number in that response category at Week 12.

Figure 2. Response Shift From Week 12 to Week 24 of Treatment



* Percentages in this column are based on total number in that response category at Week 12.

Results *(cont.)*

- At Week 24, 91 of 401 patients (22.7%) achieved T-VASI50 or better (**Table 2; Figure 3**), which was a key secondary outcome in the clinical studies
  - Improved response from Week 24 to 52 was observed in 48.4% of patients
  - At Week 52, approximately one-third (33.7%) remained stable in responses previously attained at Week 24
  - Decreased response from Week 24 to Week 52 was observed in 3.2% of patients; 14.7% had missing evaluations
- By Week 52, 178 of 401 patients (44.4%) achieved T-VASI50 or better

Table 2. Response Shift From Week 24 to Week 52 of Treatment

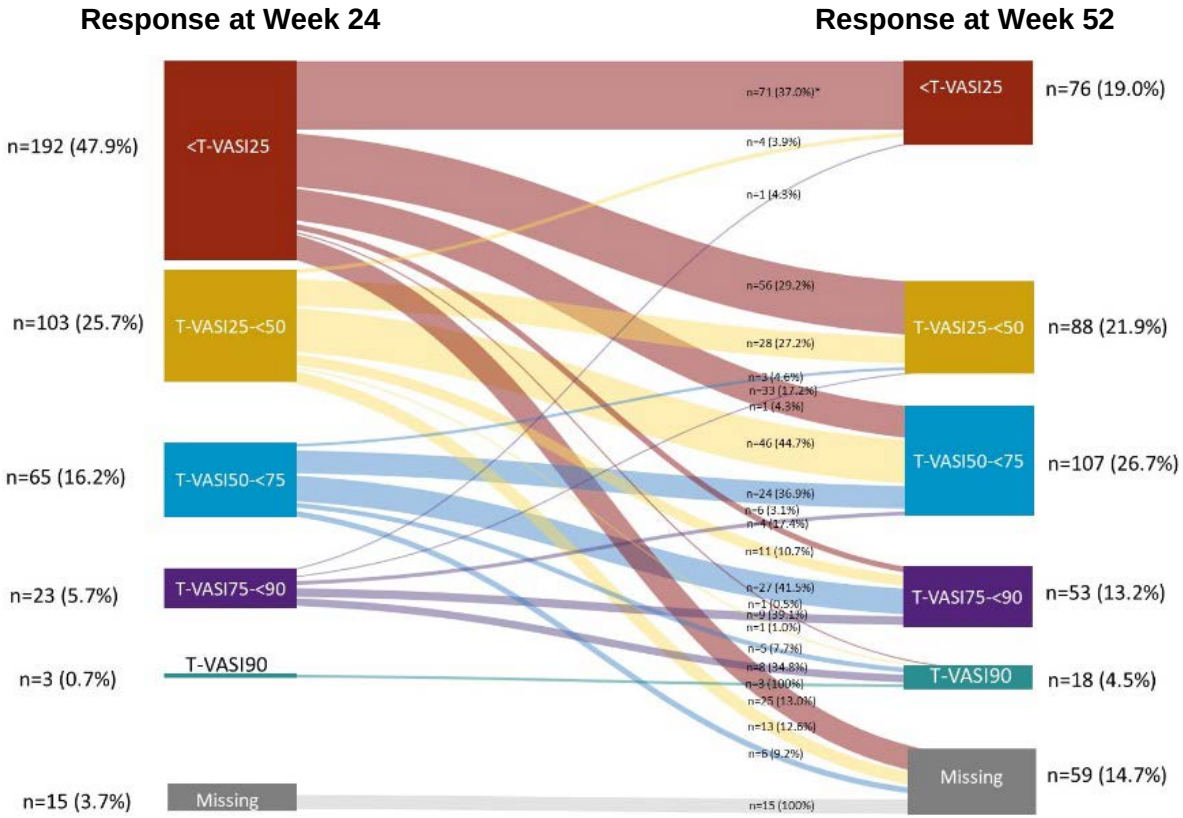
Response	Response at Week 24, n (%)*	Response at Week 52, n (%)†					
		<T-VASI25	T-VASI 25–<50	T-VASI 50–<75	T-VASI 75–<90	T-VASI ≥90	Missing
T-VASI<25	192 (47.9)	71 (37.0)	56 (29.2)	33 (17.2)	6 (3.1)	1 (0.5)	25 (13.0)
T-VASI25–<50	103 (25.7)	4 (3.9)	28 (16.0)	46 (44.7)	11 (10.7)	1 (1.0)	13 (12.6)
T-VASI50<75	65 (16.2)	0	3 (32.1)	24 (36.9)	27 (41.5)	5 (7.7)	6 (9.2)
T-VASI75–<90	23 (5.7)	1 (4.3)	1 (1.7)	4 (17.4)	9 (39.1)	8 (34.8)	0
T-VASI≥90	3 (0.7)	0	0	0	0	3 (100)	0
Missing	15 (3.7)	0	0	0	0	0	15 (100)
Total	401 (100)	76 (19.0)	88 (21.9)	107 (26.7)	53 (13.2)	18 (4.5)	59 (14.7)

T-VASI, total Vitiligo Area Scoring Index; T-VASI25, ≥25% improvement from baseline; T-VASI50, ≥50% improvement from baseline; T-VASI75, ≥75% improvement from baseline; T-VASI90, ≥90% improvement from baseline.

* Percentage based on total evaluable patients (n=401) at Week 12.

† Percentage based on total number in that response category at Week 24.

Figure 3. Response Shift From Week 24 to Week 52 of Treatment



* Percentages in this column are based on total number in that response category at Week 24.

Results *(cont.)*

Safety

- Ruxolitinib cream was well tolerated through 52 weeks of treatment
- A total of 58.6% of patients experienced ≥1 treatment-emergent adverse event (AE), most commonly COVID-19 (7.3%), application site acne (6.5%), nasopharyngitis (5.8%), and application site pruritus (5.3%)
- Application site reactions were the most common treatment-related AEs; these included acne (5.6%) and pruritus (4.7%), all of which were mild or moderate
- No serious AEs were considered related to treatment
- There were no clinically significant changes in hemoglobin or platelet levels

Conclusions

- **Adolescents and adults with nonsegmental vitiligo applying ruxolitinib cream largely improved or remained stable in their T-VASI responses from Weeks 12 to 24 and from Weeks 24 to 52 per pooled data from the TRuE-V1/TRuE-V2 phase 3 studies**
  - **A response of T-VASI50 or better was attained by 10.7% of patients at Week 12, 22.7% at Week 24, and 44.4% at Week 52**
- **Ruxolitinib was well tolerated, with no serious treatment-related AEs reported through 52 weeks**

Disclosures

DR has received honoraria as a consultant for AbbVie, Abcuro, AltruBio, Boehringer Ingelheim, Bristol Myers Squibb, Celgene, Concert, Dermavant Sciences, Dermira, Incyte, Janssen, Kyowa Kirin, Lilly, Novartis, Pfizer, Regeneron Pharmaceuticals, Revolo Biotherapeutics, Sanofi, Sun Pharmaceuticals, UCB, and Viela Bio; has received research support from AbbVie, Amgen, Bristol Myers Squibb, Celgene, Dermira, Galderma, Incyte, Janssen, Lilly, Merck, Novartis, Pfizer, and Regeneron Pharmaceuticals; and has served as a paid speaker for AbbVie, Amgen, Celgene, Janssen, Lilly, Novartis, Pfizer, Regeneron Pharmaceuticals, and Sanofi. JEH has served as a consultant for AbbVie, Aclaris Therapeutics, BiologicsMD, EMD Serono, Genzyme/Sanofi, Janssen, Pfizer, Rheos Medicines, Sun Pharmaceuticals, TeVido BioDevices, The Expert Institute, 3rd Rock Ventures, and Villaris Therapeutics; has served as an investigator for Aclaris Therapeutics, Celgene, Dermira, EMD Serono, Genzyme/Sanofi, Incyte Corporation, LEO Pharma, Pfizer, Rheos Medicines, Stiefel/GlaxoSmithKline, Sun Pharmaceuticals, TeVido BioDevices, and Villaris Therapeutics; holds equity in Aldena Therapeutics, NIRA Biosciences, Rheos Medicines, TeVido BioDevices, and Villaris Therapeutics; is a scientific founder of Aldena Therapeutics, NIRA Biosciences, and Villaris Therapeutics; and has patents pending for IL-15 blockade for treatment of vitiligo, JAK inhibition with light therapy for vitiligo, and CXCR3 antibody depletion for treatment of vitiligo. AW is a dermatologist at the Netherlands Institute for Pigment Disorders and the Department of Dermatology at the Amsterdam University Medical Center; has served as principal investigator for Avita Medical, Incyte, and Novartis; has served as an advisory board member for Incyte; has received research grants from Avita Medical and Lumenis; and has received devices from Humeca and PerfAction. ML is an employee of Mount Sinai Hospital, which receives research funds from AbbVie, Amgen, Arcutis, Avotres, Boehringer Ingelheim, Dermavant, Eli Lilly, Incyte, Janssen Research & Development, LLC, Ortho Dermatologics, Regeneron, and UCB, Inc; and is a consultant for Aditum Bio, Almirall, AnaptysBio, Arcutis, Aristea, Arrive Technology, Avotres Therapeutics, BioMX, Boehringer Ingelheim, Bristol Myers Squibb, Cara Therapeutics, Castle Biosciences, Corrona, Dermavant Sciences, Dr. Reddy's Laboratories, Evelo, Evommune, Facilitate International Dermatologic Education, Forte, Foundation for Research and Education in Dermatology, Helsinn, Incyte, LEO Pharma, Meiji Seika Pharma, Mindera, Pfizer, and Verrica. KE is a consultant for AbbVie, Incyte, La Roche-Posay, Pfizer, Pierre Fabre, Sanofi, and Viela Bio. TP has received grants and/or honoraria from AbbVie, ACM Pharma, Almirall, Amgen, Astellas, Bristol Myers Squibb, Celgene, Galderma, Genzyme/Sanofi, GlaxoSmithKline, Incyte, Janssen, LEO Pharma, Eli Lilly, Novartis, Pfizer, Sun Pharmaceuticals, and UCB; is the cofounder of YUKIN Therapeutics; and has patents on WNT agonists or GSK3b antagonists for repigmentation of vitiligo and on the use of CXCR3B blockers in vitiligo. DK, HR, and KB are employees and shareholders of Incyte. JS has received grants and/or honoraria from AbbVie, Bristol Myers Squibb, Calypso Biotech, Eli Lilly, Incyte, LEO Pharma, Novartis, Pfizer, Pierre Fabre, Sanofi, Sun Pharmaceuticals, and Viela Bio; and has patents on MMP9 inhibitors and uses thereof in the prevention or treatment of a depigmenting disorder and three-dimensional model of depigmenting disorder.

Acknowledgments

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