Correlation of Patient-Reported Vitiligo Noticeability Scale With Physician-Assessed Facial Vitiligo Area Scoring Index Responses: Post Hoc Analyses From the TRuE-V Trials of Ruxolitinib Cream in Vitiligo

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Introduction

- Vitiligo is a chronic autoimmune disease that targets melanocytes, resulting in patches of skin depigmentation[1]
- A cream formulation of ruxolitinib, a Janus kinase (JAK) JAK1/2 inhibitor, is approved in the United States, Europe, and the United Kingdom for topical treatment of nonsegmental vitiligo in patients aged ≥12 years[2-4]
- In two 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[5]

Objectives

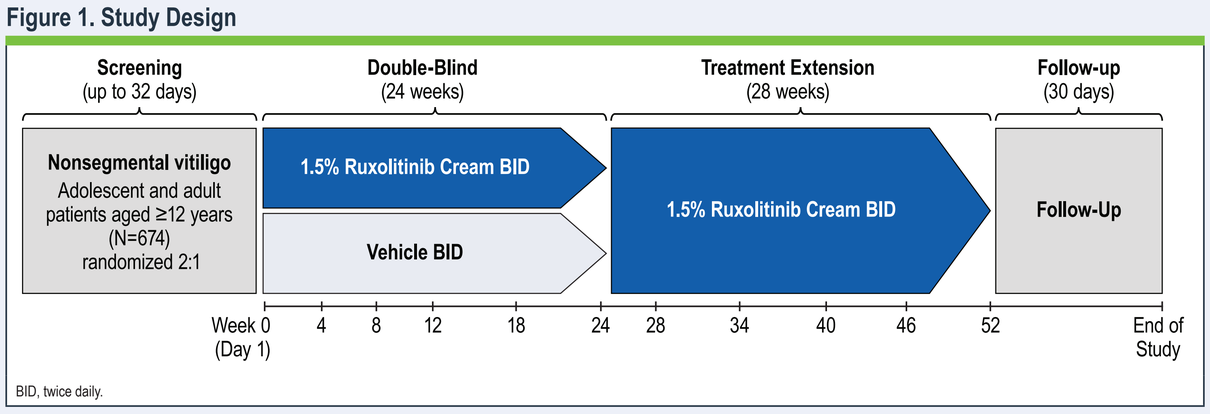
- We present post hoc Vitiligo Noticeability Scale (VNS) and facial Vitiligo Area Scoring Index (F-VASI) correlation data for patients who applied 1.5% ruxolitinib cream twice daily from Day 1 in the TRuE-V trials

Methods

Patients and Study Design

- The study design and eligibility criteria for the parent studies (TRuE-V1/TRuE-V2) have been described previously[5]
  - Patients ≥12 years old diagnosed with nonsegmental vitiligo with depigmentation covering ≤10% of total body surface area (BSA) were randomized 2:1 to apply 1.5% ruxolitinib cream twice daily or vehicle twice daily for 24 weeks (**Figure 1**)
  - After 24 weeks of treatment, all patients could apply 1.5% ruxolitinib cream twice daily for an additional 28 weeks (through Week 52) in the open-label treatment extension

Figure 1. Study Design



BID, twice daily.

Endpoints and Analysis

- The VNS is a patient-reported measure of treatment success; patients were shown a baseline photograph and provided a mirror to assess how noticeable their facial vitiligo was at the study visit compared with baseline[6]
  - VNS scores were assessed on a 5-point scale (1-more noticeable, 2-as noticeable, 3-slightly less noticeable, 4-a lot less noticeable, 5-no longer noticeable) and 2 levels of response were defined for analysis: VNS 3/4/5 and VNS 4/5
- F-VASI response was assessed by physicians as a ≥50%, ≥75%, or ≥90% improvement from baseline (F-VASI50, F-VASI75, and F-VASI90, respectively)
- Pooled data for patients who had both VNS and F-VASI evaluations at Weeks 24 and 52 were categorized at Week 24 and Week 52 by VNS responses (VNS 3/4/5 and VNS 4/5) and F-VASI responses (F-VASI50, F-VASI75, and F-VASI90)

Statistical Analysis

- VNS response was correlated with F-VASI response by tetrachoric (Rtet; for binary purposes) and polyserial correlations (for ordinal responses), and associated *P* values were calculated

Results

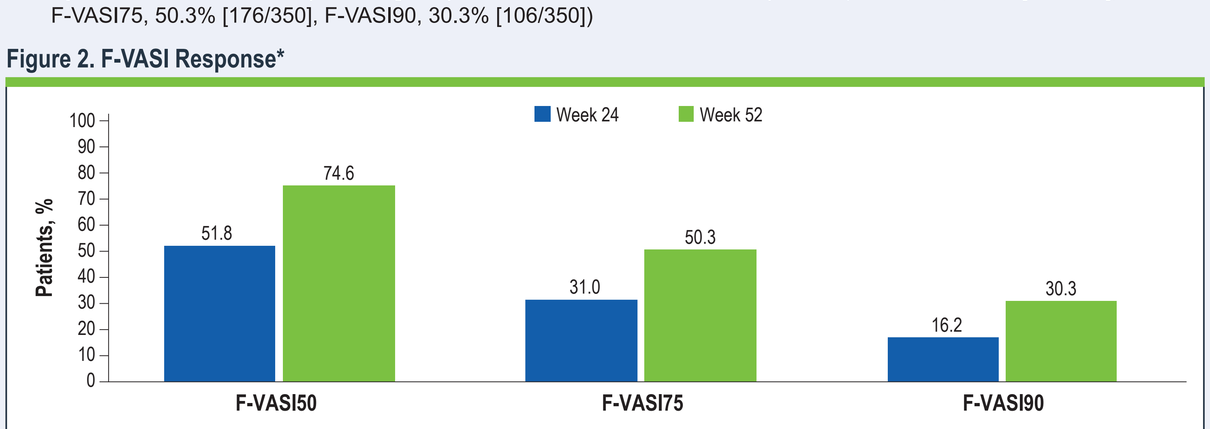
Patients

- A total of 674 patients were randomized in the TRuE-V1/TRuE-V2 trials (ruxolitinib cream, n=450; vehicle, n=224)
  - Mean (SD) age was 39.6 (15.1) years and disease duration was 14.8 (11.6) years
  - Mean (SD) facial and total BSA at baseline was 1.02% (0.64%) and 7.39% (2.03%), respectively

F-VASI Response

- In total, 394 patients had VNS and F-VASI evaluations at Week 24, and 350 patients had VNS and F-VASI evaluations at Week 52
- Among those patients with evaluations at Week 24, F-VASI50, F-VASI75, and F-VASI90 were achieved by 51.8% (204/394), 31.0% (122/394), and 16.2% (64/394), respectively (**Figure 2**)
- By Week 52, an increased percentage of patients had achieved F-VASI responses (F-VASI50, 74.6% [261/350]; F-VASI75, 50.3% [176/350], F-VASI90, 30.3% [106/350])

Figure 2. F-VASI Response*



* Among 394 patients at Week 24 and 390 patients at Week 52 with VNS and F-VASI evaluations.
F-VASI, facial Vitiligo Area Scoring Index; F-VASI50/75/90, F-VASI improvement from baseline of ≥50%/≥75%/≥90%.

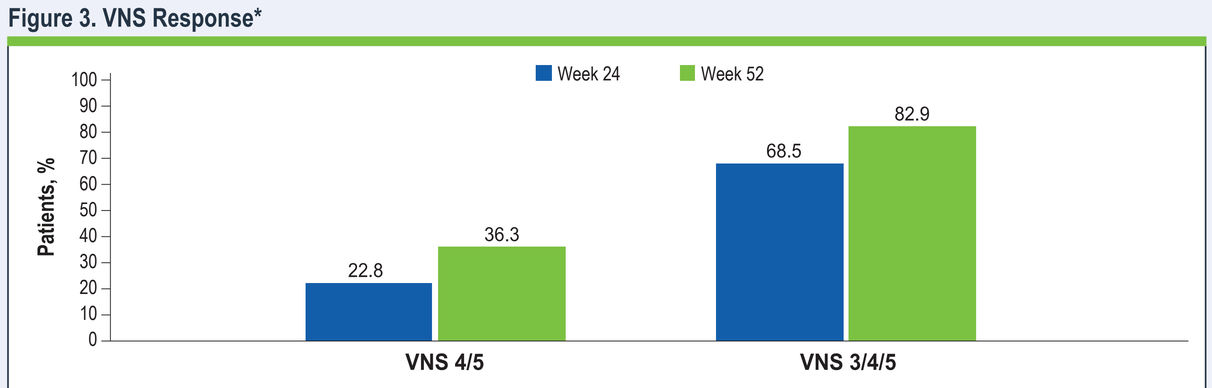
VNS Response

- Among the 394 patients with VNS and F-VASI evaluations at Week 24, a VNS 3/4/5 response was achieved by 68.5% of patients (270/394) and VNS 4/5 response was achieved by 22.8% (90/394; **Figure 3**)
- By Week 52, response rates had increased to 82.9% (290/350) for VNS 3/4/5 and to 36.3% (127/350) for VNS 4/5

VNS 4/5 and F-VASI Response Correlation

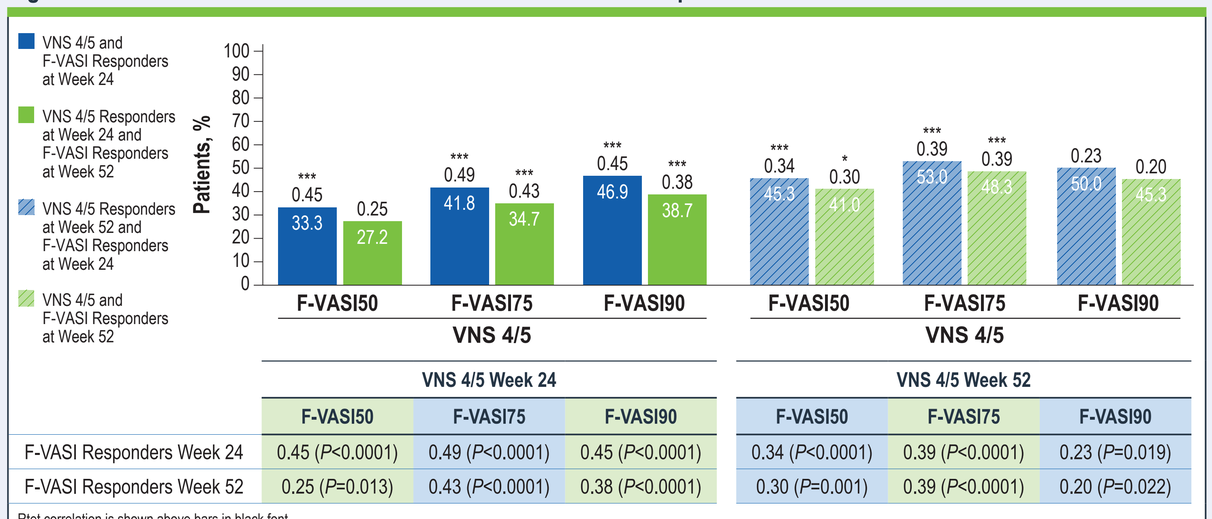
- A VNS 4/5 response at Week 24 was weakly/moderately (Rtet, <0.50) but significantly correlated (*P*<0.0001) with F-VASI50, F-VASI75, and F-VASI95 at Week 24 and with F-VASI75 and F-VASI90 at Week 52 (**Figure 4**)
- A VNS 4/5 response at Week 52 was weakly/moderately (Rtet, <0.40) but significantly correlated (*P*<0.0001) with F-VASI50 and F-VASI75 at Week 24 and with F-VASI50 (*P*=0.001) and F-VASI75 (*P*<0.0001) at Week 52
- The strongest correlations were observed for VNS 4/5 and F-VASI75 responses at Week 24 (Rtet, 0.49; *P*<0.0001), indicating a moderately positive association between variables

Figure 3. VNS Response*



* Among 394 patients at Week 24 and 390 patients at Week 52 with VNS and F-VASI evaluations.
VNS, Vitiligo Noticeability Scale; VNS 3/4/5, VNS response of a lot less noticeable/slightly less noticeable/no longer noticeable compared with baseline; VNS 4/5, VNS response of a lot less noticeable/no longer noticeable compared with baseline.

Figure 4. Tetrachoric Correlation Between VNS 4/5 and F-VASI Responses



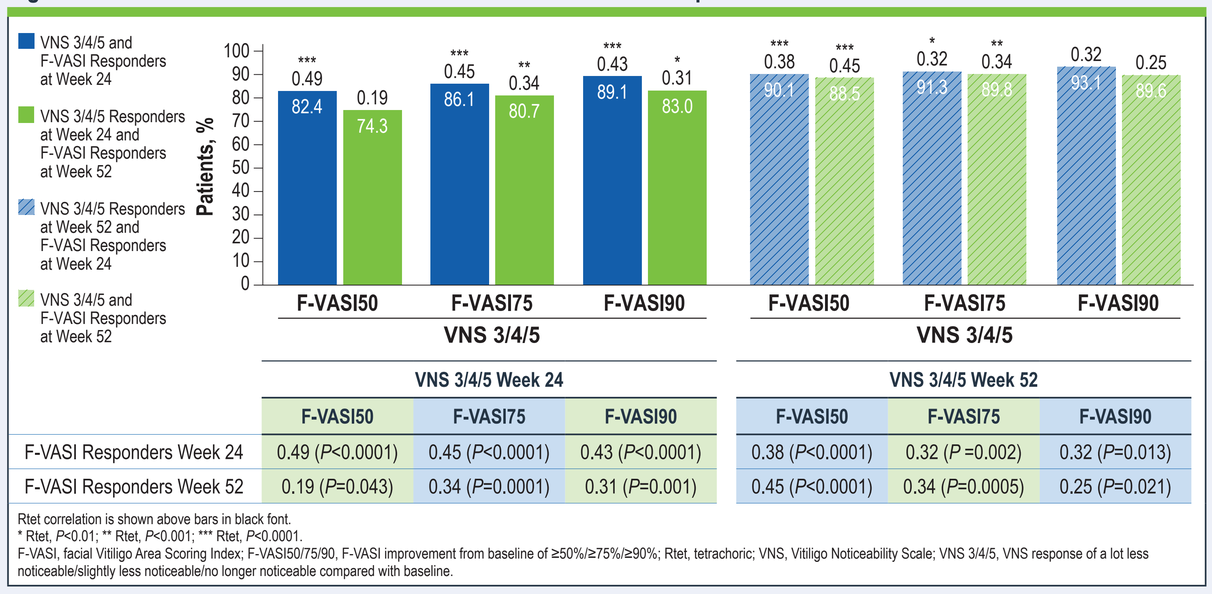
	VNS 4/5 Week 24			VNS 4/5 Week 52		
	F-VASI50	F-VASI75	F-VASI90	F-VASI50	F-VASI75	F-VASI90
F-VASI Responders Week 24	0.45 (*P*<0.0001)	0.49 (*P*<0.0001)	0.45 (*P*<0.0001)	0.34 (*P*<0.0001)	0.39 (*P*<0.0001)	0.23 (*P*=0.019)
F-VASI Responders Week 52	0.25 (*P*=0.013)	0.43 (*P*<0.0001)	0.38 (*P*<0.0001)	0.30 (*P*=0.001)	0.39 (*P*<0.0001)	0.20 (*P*=0.022)

Rtet correlation is shown above bars in black font.
* Rtet, *P*<0.01; ** Rtet, *P*<0.001; *** Rtet, *P*<0.0001.
F-VASI, facial Vitiligo Area Scoring Index; F-VASI50/75/90, F-VASI improvement from baseline of ≥50%/≥75%/≥90%; Rtet, tetrachoric; VNS, Vitiligo Noticeability Scale; VNS 4/5, VNS response of a lot less noticeable/no longer noticeable compared with baseline.

VNS 3/4/5 and F-VASI Response Correlation

- A VNS 3/4/5 response at Week 24 was weakly/moderately (Rtet, <0.50) but significantly correlated (*P*<0.0001) with F-VASI50, F-VASI75, and F-VASI95 at Week 24 and with F-VASI75 (*P*=0.0001) and F-VASI90 (*P*=0.001) at Week 52 (**Figure 5**)
- A VNS 3/4/5 response at Week 52 was weakly/moderately (Rtet, <0.50) but significantly correlated with F-VASI50 (*P*<0.0001) and F-VASI75 (*P*=0.002) at Week 24 and with F-VASI50 (*P*<0.0001) and F-VASI75 (*P*=0.0005) at Week 52
- The strongest correlations were observed for VNS 3/4/5 and F-VASI50 responses at Week 24 (Rtet, 0.49; *P*<0.0001), indicating a moderately positive association between variables

Figure 5. Tetrachoric Correlation Between VNS 3/4/5 and F-VASI Responses



	VNS 3/4/5 Week 24			VNS 3/4/5 Week 52		
	F-VASI50	F-VASI75	F-VASI90	F-VASI50	F-VASI75	F-VASI90
F-VASI Responders Week 24	0.49 (*P*<0.0001)	0.45 (*P*<0.0001)	0.43 (*P*<0.0001)	0.38 (*P*<0.0001)	0.32 (*P* =0.002)	0.32 (*P*=0.013)
F-VASI Responders Week 52	0.19 (*P*=0.043)	0.34 (*P*=0.0001)	0.31 (*P*=0.001)	0.45 (*P*<0.0001)	0.34 (*P*=0.0005)	0.25 (*P*=0.021)

Rtet correlation is shown above bars in black font.
* Rtet, *P*<0.01; ** Rtet, *P*<0.001; *** Rtet, *P*<0.0001.
F-VASI, facial Vitiligo Area Scoring Index; F-VASI50/75/90, F-VASI improvement from baseline of ≥50%/≥75%/≥90%; Rtet, tetrachoric; VNS, Vitiligo Noticeability Scale; VNS 3/4/5, VNS response of a lot less noticeable/slightly less noticeable/no longer noticeable compared with baseline.

Conclusions

- **This pooled post hoc analysis of patients from the TRuE-V trials treated with ruxolitinib cream showed that the quality and patients' perception of repigmentation as reflected by patient-reported VNS was moderately but significantly associated with physician-assessed F-VASI clinical outcomes**
- **These findings suggest that patient-reported VNS response does not necessarily predict physician-reported F-VASI response; therefore, both tools are important to assess outcomes since they measure different constructs of interest**

Disclosures

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 Corporation, and Novartis; has served as an advisory board member for Incyte Corporation; has received research grants from Avita Medical and Lumenis; and has received devices from Humeca and PerfAction. TP has received grants and/or honoraria from AbbVie, ACM Pharma, Almirall, Amgen, Astellas, Bristol Myers Squibb, Calypso, Celgene, Galderma, Genzyme/Sanofi, GlaxoSmithKline, Incyte, Janssen, LEO Pharma, Eli Lilly, Novartis, Pfizer, Sun Pharmaceuticals, UCB, and Vyne Therapeutics; is the cofounder of YUKIN Therapeutics; and has patents on WNT agonists or GSK3b antagonist for repigmentation of vitiligo and on the use of CXCR3B blockers in vitiligo. 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. MS has received fees for consultancy and/or lectures and/or implementation of clinical studies for: AbbVie, Affibody, Amgen, Almirall, Aristea, Astra Zeneca, Boehringer Ingelheim, Bristol Myers Squibb, Celgene, Dermira, Dr. August Wolff, Dr. Reddy's Laboratories, Eli Lilly, Galderma, Genentech, GlaxoSmithKline, Incyte, Janssen-Cilag, LEO Pharma, MedImmune, Menlo Therapeutics, MSD, Mundipharma, Medac, Moonlake, Novartis, Pfizer, Regeneron, Sanofi Genzyme and UCB Pharma. SRD has received fees and/or honoraria as a consultant for Almirall, Avita, Bristol Myers Squibb, Cassiopea SpA, Dermavant Sciences, Dermira, Ferndale Laboratories, Foamix, Galderma Laboratories LP, Incyte, MC2 Therapeutics, Ortho Dermatologics, Pfizer, Scientis, Sente Labs, SkinCeuticals LLC, UCB, and Verrica Pharmaceuticals; has received stock options as a consultant for Gore Range Capital; has received honoraria as a speaker for Almirall and Ortho Dermatologics; has received grants/research funding as an investigator for AbbVie, AOBiome LLC, Atacama Therapeutics, Brickell Biotech, Dermavant Sciences, Incyte, Novan, and SkinMedica; has served as an advisory board member for the Foundation for Research & Education of Dermatology; is a stockholder of Gore Range Capital; and is a shareholder in PDP of Texas. MSA is a principal investigator for Incyte working on vitiligo clinical trials. 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. KButler and KBibeau were employees and shareholders of Incyte at the time of the study. HR is an employee and shareholder of Incyte. AGP has served as an investigator for Aclaris Therapeutics, Immune Tolerance Network, Incyte, and Pfizer; a consultant for AbbVie, Arcutis, Avita Medical, Chromaderm, Immune Tolerance Network, Incyte, Pfizer, Thalocan, TWi, Viela Bio, Vimela, Villaris, Vyne and WCG/Trifecta; and holds stock options for Tara Medical and Zerigo Health.

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