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Facial and Total Vitiligo Area Scoring Index Response Shift During 104 Weeks of Ruxolitinib Cream Treatment for Vitiligo: Results From the Open-Label Arm of the TRuE-V Long-Term Extension Phase 3 Study

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Presenting Author Disclosures

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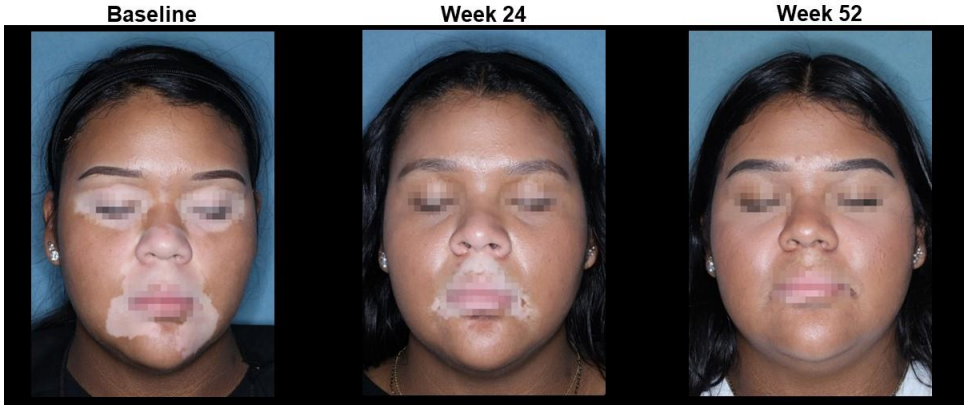
JAK-Targeted Therapy for Vitiligo

- Vitiligo is a chronic autoimmune disease that destroys melanocytes, leading to skin depigmentation[1]
- Ruxolitinib cream, a JAK1/JAK2 inhibitor in a topical formulation, is approved by the US FDA for the treatment of nonsegmental vitiligo in adult and pediatric patients ≥12 years of age,[2] a milestone in vitiligo therapy
- 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, with continued improvement in outcomes through Week 52[3]

Patient Who Achieved F-VASI90 at Week 52 in the TRuE-V Studies



FDA, US Food and Drug Administration; F-VASI90, ≥90% improvement from baseline in facial Vitiligo Area Scoring Index; JAK, Janus kinase; TRuE-V, Topical Ruxolitinib Evaluation in Vitiligo trials.

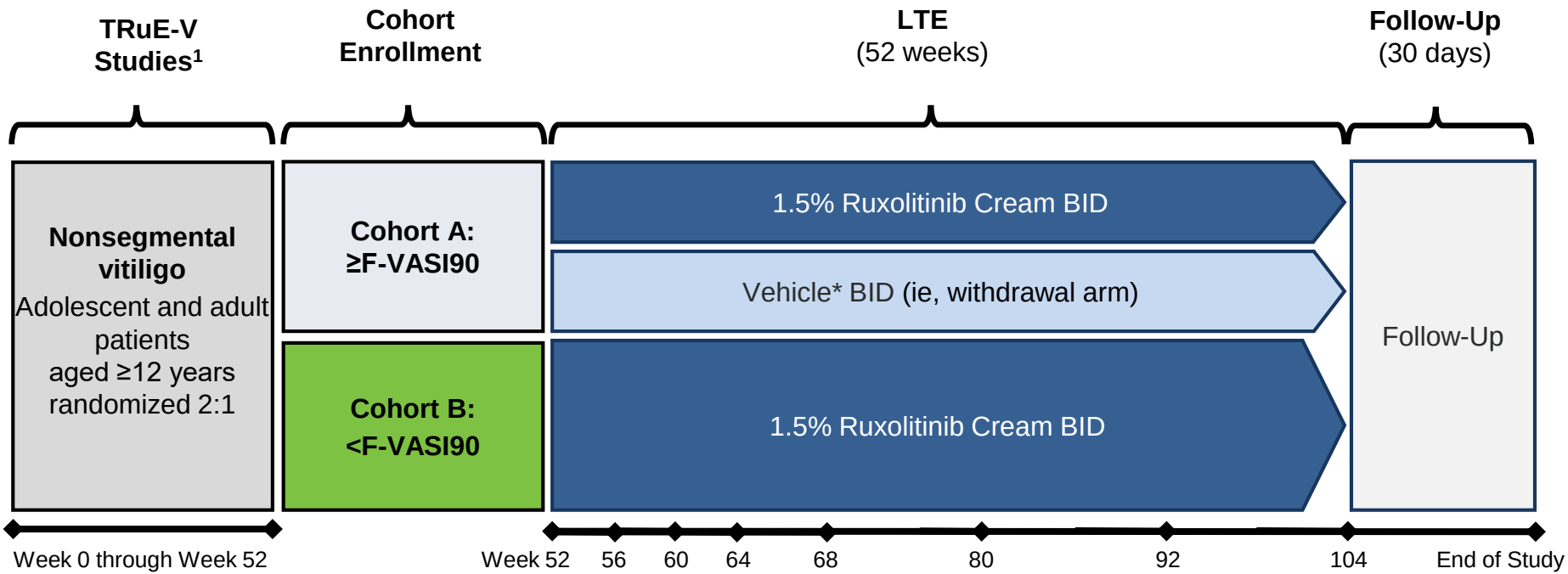
1. Rodrigues M, et al. *J Am Acad Dermatol*. 2017;77:1-13; 2. OPZELURA™ (ruxolitinib) cream). Full Prescribing Information. Incyte Corporation; July 2022; 3. Rosmarin D, et al. *N Engl J Med.* 2022;387:1445-1455.

Objective

- To evaluate the change (or stability) in F-VASI and T-VASI response categories in patients with nonsegmental vitiligo enrolled in the TRuE-V LTE study (NCT04530344) who:
  - Did not achieve almost complete repigmentation (ie, F-VASI90) at Week 52 in the TRuE-V1/TRuE-V2 phase 3 studies (Cohort B) and
  - Continued to apply ruxolitinib cream through Week 104

F-VASI, facial Vitiligo Area Scoring Index; F-VASI90; ≥90% improvement from baseline in F-VASI; LTE, long-term extension; T-VASI, total Vitiligo Area Scoring Index.

TRuE-V LTE Study Design



BID, twice daily.
* Patients randomized to vehicle who relapsed (ie, <F-VASI75) could apply 1.5% ruxolitinib cream BID rescue treatment for the remainder of the LTE period.
1. Rosmarin D, et al. *N Engl J Med.* 2022;387:1445-1455.

TRuE-V LTE Cohort B Study Endpoints

- **Efficacy Endpoints**
  - Percentage of patients applying ruxolitinib cream from Day 1 or switching from vehicle to ruxolitinib cream at Week 24 of the TRuE-V phase 3 studies who did not achieve almost complete facial repigmentation (ie, F-VASI90) at Week 52 achieving the following outcomes at Week 104:
    - F-VASI75
    - F-VASI90
    - ≥50% improvement from baseline in T-VASI (T-VASI50)
  - Maintenance and shift in F-VASI and T-VASI responses at Weeks 52, 80, and 104
- **Safety and tolerability were also assessed**

F-BSA, facial body surface area; VNS, Vitiligo Noticeability Scale.

Patient Demographics

Cohort B (<F-VASI90 at Week 52)

- Baseline demographics and clinical characteristics were similar between treatment groups

Characteristic	Ruxolitinib cream from Day 1 (n=224)	Vehicle to ruxolitinib cream (n=118)	Overall (N=342)
Age, median (IQR), y	39.0 (26.0–51.0)	39.0 (30.0–49.0)	39.0 (27.0–51.0)
Female, n (%)	129 (57.6)	61 (51.7)	190 (55.6)
White, n (%)	180 (80.4)	107 (90.7)	287 (83.9)
Fitzpatrick skin type, n (%)			
I	6 (2.7)	1 (0.8)	7 (2.0)
II	51 (22.8)	45 (38.1)	96 (28.1)
III	106 (47.3)	51 (43.2)	157 (45.9)
IV	43 (19.2)	14 (11.9)	57 (16.7)
V	13 (5.8)	6 (5.1)	19 (5.6)
VI	5 (2.2)	1 (0.8)	6 (1.8)
Baseline F-VASI, mean (SD)	0.91 (0.55)	0.88 (0.54)	0.90 (0.55)
Baseline T-VASI, mean (SD)	6.73 (2.02)	6.70 (2.15)	6.72 (2.06)

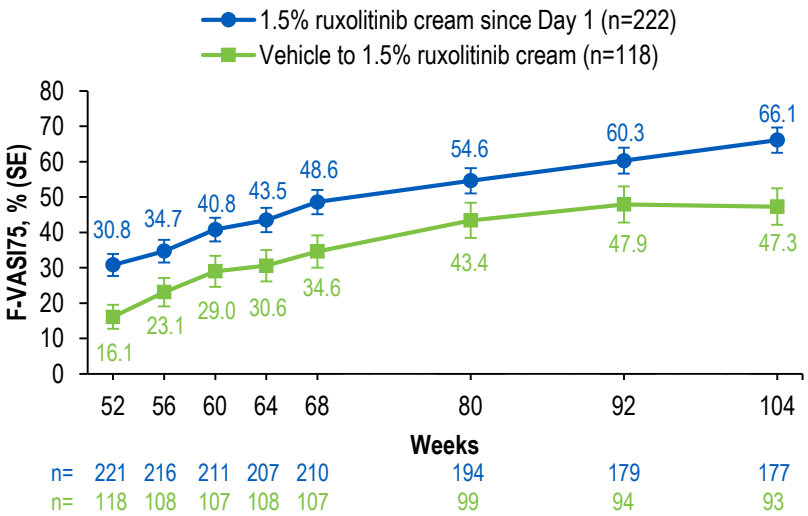
Characteristic	Ruxolitinib cream from Day 1 (n=224)	Vehicle to ruxolitinib cream (n=118)	Overall (N=342)
F-BSA,* mean (SD), %	1.02 (0.64)	1.01 (0.63)	1.02 (0.63)
T-BSA, mean (SD), %	7.50 (2.00)	7.42 (2.06)	7.47 (2.02)
Duration of disease, median (IQR), y	11.7 (5.2–21.7)	13.6 (6.8–23.4)	12.2 (5.7–21.9)
Diagnosed in childhood, n (%)	86 (38.4)	43 (36.4)	129 (37.7)
Disease stability,† n (%)			
Stable	167 (74.6)	91 (77.1)	258 (75.4)
Progressive	57 (25.4)	27 (22.9)	84 (24.6)
Other autoimmune disorders, n (%)	42 (18.8)	26 (22.0)	68 (19.9)
Previous therapy,‡ n (%)	138 (61.6)	69 (58.5)	207 (60.5)
Topical calcineurin inhibitor	79 (35.3)	38 (32.2)	117 (34.2)
Topical corticosteroid	72 (32.1)	24 (20.3)	96 (28.1)
Phototherapy§	70 (31.3)	37 (31.4)	107 (31.3)

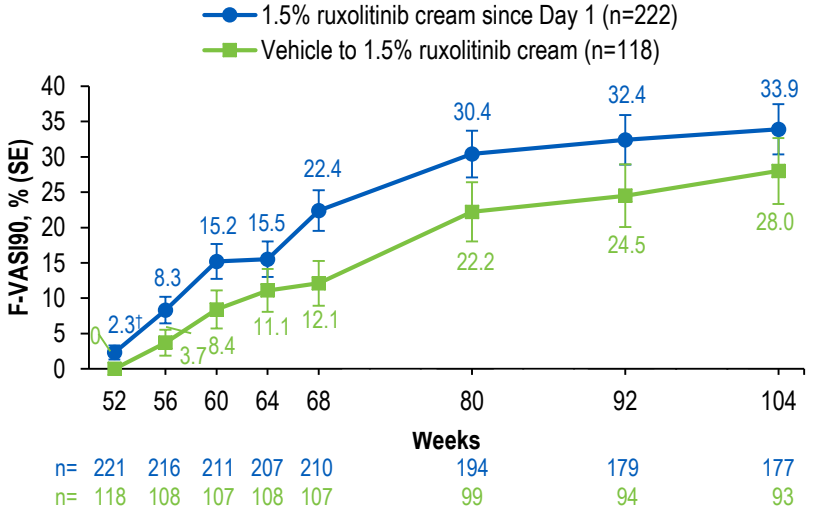
F-BSA, facial body surface area; IQR, interquartile range; NB-UVB, narrow-band ultraviolet-B; PUVA, psoralen ultraviolet-A; T-BSA, total body surface area.
* Percentage of T-BSA. † Determination of disease stability was based on investigator judgment. ‡ Patients could have used multiple previous lines of therapy. § Phototherapy includes NB-UVB phototherapy, excimer laser, PUVA photochemotherapy, and other phototherapy.

F-VASI Responses at Weeks 52–104

Cohort B (<F-VASI90 at Week 52)

- 66.1% of patients who applied ruxolitinib cream since Day 1 achieved F-VASI75 at Week 104 (LTE end-of-treatment), increasing from 30.8% at Week 52 (LTE baseline) and 54.6% at Week 80
  - 33.9% of patients applying ruxolitinib cream since Day 1 attained F-VASI90 at Week 104
  - Percentages of patients who switched from vehicle to ruxolitinib cream after Week 24 and achieved F-VASI75 and F-VASI90 also increased during the LTE period



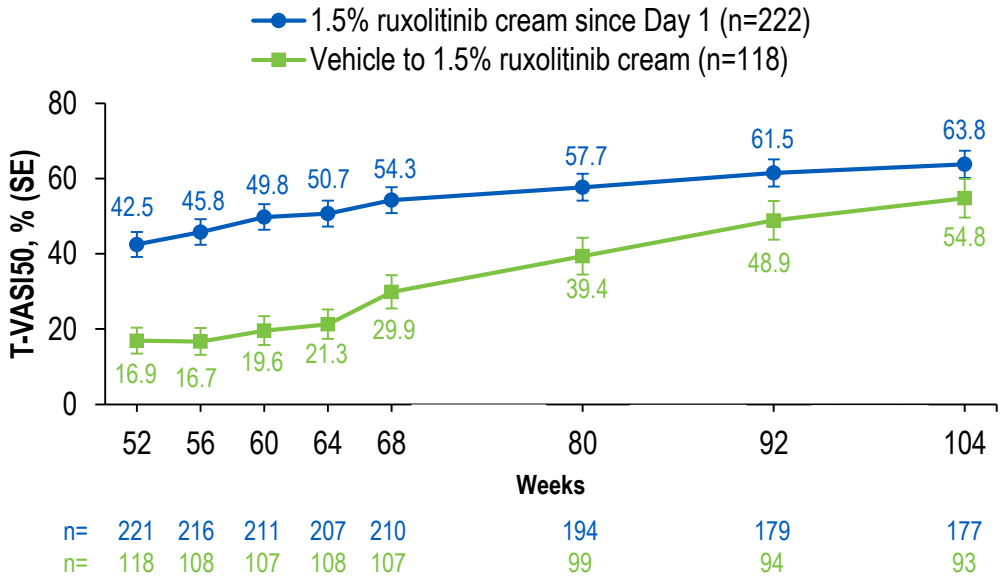


† Five patients were incorrectly assigned to Cohort B at LTE baseline.

T-VASI Response at Weeks 52–104

Cohort B (<F-VASI90 at Week 52)

- T-VASI50 was achieved by 63.8% of patients applying ruxolitinib cream since Day 1, increasing from 42.5% at Week 52 and 57.7% at Week 80
  - The percentage patients who switched from vehicle to ruxolitinib cream after Week 24 who also attained T-VASI50 increased similarly throughout the LTE period



Maintenance and Shift in F-VASI Response at Weeks 80 & 104: Ruxolitinib Cream from Day 1

- At Week 80, 47.1% (104/221) improved in F-VASI responses previously attained at Week 52; 36.2% (80/221) remained stable
- At Week 104, 19.6% (38/194) improved in F-VASI responses attained at Week 80; 64.4% (125/194) remained stable
- Rates of F-VASI maintenance and improvement were similar among patients who switched from vehicle to 1.5% ruxolitinib cream at Week 24

Response	Week 52 Response, n (%)*	Week 80 Response, n (%)†					
		<F-VASI25	F-VASI 25–<50	F-VASI 50–<75	F-VASI 75–<90	F-VASI90	Missing
<F-VASI25	41 (18.5)	15 (36.6)	9 (22.0)	1 (2.4)	1 (2.4)	4 (9.8)	11 (26.8)
F-VASI 25–<50	35 (15.8)	1 (2.9)	9 (25.7)	13 (37.1)	4 (11.4)	4 (11.4)	4 (11.4)
F-VASI 50–<75	77 (34.7)	1 (1.3)	4 (5.2)	31 (40.3)	22 (28.6)	15 (19.5)	4 (5.2)
F-VASI 75–<90	63 (28.4)	2 (3.2)	0	2 (3.2)	20 (31.7)	31 (49.2)	8 (12.7)
F-VASI90	5 (2.3)	0	0	0	0	5 (100)	0
Missing	1 (0.5)	0	0	0	0	0	1 (100)
Total	**222 (100)**	**19 (8.6)**	**22 (9.9)**	**47 (21.2)**	**47 (21.2)**	**59 (26.6)**	**28 (12.6)**

Response	Week 80 Response, n (%)*	Week 104 Response, n (%)†					
		<F-VASI25	F-VASI 25–<50	F-VASI 50–<75	F-VASI 75–<90	F-VASI90	Missing
<F-VASI25	19 (8.6)	11 (57.9)	0	0	3 (15.8)	0	5 (26.3)
F-VASI 25–<50	22 (9.9)	1 (4.5)	8 (36.4)	6 (27.3)	3 (13.6)	1 (4.5)	3 (13.6)
F-VASI 50–<75	47 (21.2)	0	1 (2.1)	26 (55.3)	13 (27.7)	5 (10.6)	2 (4.3)
F-VASI 75–<90	47 (21.2)	0	1 (2.1)	2 (4.3)	33 (70.2)	7 (14.9)	4 (8.5)
F-VASI90	59 (26.6)	0	0	2 (3.4)	5 (8.5)	47 (79.7)	5 (8.5)
Missing	28 (12.6)	2 (7.1)	0	0	0	0	26 (92.9)
Total	**222 (100)**	**14 (6.3)**	**10 (4.5)**	**36 (16.2)**	**57 (25.7)**	**60 (27.0)**	**45 (20.3)**

* Percentage based on total evaluable patients (n=222). † Percentage based on total number in that response category at Week 52 or Week 80.

Maintenance and Shift in T-VASI Responses at Weeks 80 & 104: Ruxolitinib Cream from Day 1

- At Week 80, 31.7% (70/221) improved in T-VASI responses previously attained at Week 52; 51.1% (113/221) remained stable
- At Week 104, 22.2% (43/194) improved T-VASI responses previously attained at Week 80; 61.3% (119/194) remained stable
- Rates of T-VASI maintenance and improvement were similar among patients who switched from vehicle to 1.5% ruxolitinib cream at Week 24

		Week 80 Response, n (%)†					
Response	Week 52 Response, n (%)*	<T-VASI25	T-VASI 25–<50	T-VASI 50–<75	T-VASI 75–<90	T-VASI90	Missing
<T-VASI25	66 (29.7)	32 (48.5)	15 (22.7)	3 (4.5)	1 (1.5)	1 (1.5)	14 (21.2)
T-VASI 25–<50	61 (27.5)	5 (8.2)	25 (41.0)	23 (37.7)	2 (3.3)	1 (1.6)	5 (8.2)
T-VASI 50–<75	67 (30.2)	1 (1.5)	3 (4.5)	39 (58.2)	16 (23.9)	3 (4.5)	5 (7.5)
T-VASI 75–<90	22 (9.9)	0	1 (4.5)	1 (4.5)	14 (63.6)	5 (22.7)	1 (4.5)
T-VASI90	5 (2.3)	0	0	0	0	3 (60.0)	2 (40.0)
Missing	1 (0.5)	0	0	0	0	0	1 (100)
Total	**222 (100)**	**38 (17.1)**	**44 (19.8)**	**66 (29.7)**	**33 (14.9)**	**13 (5.9)**	**28 (12.6)**

		Week 104 Response, n (%)†					
Response	Week 80 Response, n (%)*	<T-VASI25	T-VASI 25–<50	T-VASI 50–<75	T-VASI 75–<90	T-VASI90	Missing
<F-VASI25	38 (17.1)	23 (60.5)	8 (21.1)	3 (7.9)	0	0	4 (10.5)
T-VASI 25–<50	44 (19.8)	4 (9.1)	22 (50.0)	11 (25.0)	1 (2.3)	0	6 (13.6)
T-VASI 50–<75	66 (29.7)	1 (1.5)	4 (6.1)	41 (62.1)	14 (21.2)	0	6 (9.1)
T-VASI 75–<90	33 (14.9)	0	1 (3.0)	2 (6.1)	22 (66.7)	6 (18.2)	2 (6.1)
T-VASI90	13 (5.9)	0	0	1 (7.7)	0	11 (84.6)	1 (7.7)
Missing	28 (12.6)	0	1 (3.6)	1 (3.6)	0	0	26 (92.9)
Total	**222 (100)**	**28 (12.6)**	**36 (16.2)**	**59 (26.6)**	**37 (16.7)**	**17 (7.7)**	**45 (20.3)**

* Percentage based on total evaluable patients (n=222). † Percentage based on total number in that response category at Week 52 or Week 80.

Safety

TEAEs in Cohort B (<F-VASI90 at Week 52) Through Week 104

- Ruxolitinib cream was well tolerated
- Treatment-related TEAEs among patients who applied ruxolitinib cream at any time were all mild or moderate (none serious)
  - Application site pruritus (n=4; 1.2%) was the most common treatment-related TEAE
  - Application site acne was reported in 6 patients (1.8%; 1 [0.3%] was treatment-related)

Characteristic, n (%)	Ruxolitinib cream from Day 1 (n=224)	Vehicle to ruxolitinib cream (n=118)	Overall (N=342)
Patients with TEAE	114 (50.9)	59 (50.0)	173 (50.6)
Most common TEAEs†			
COVID-19	34 (15.2)	11 (9.3)	45 (13.2)
Nasopharyngitis	11 (4.9)	5 (4.2)	16 (4.7)
Upper respiratory tract infection	4 (1.8)	6 (5.1)	10 (2.9)
Urinary tract infection	7 (3.1)	1 (0.8)	8 (2.3)
Viral infection	1 (0.4)	4 (3.4)	5 (1.5)
Patients with treatment-related TEAE	14 (6.3)	6 (5.1)	20 (5.8)
Patients with application site reaction	19 (8.5)	6 (5.1)	25 (7.3)
Patients with serious TEAE‡	7 (3.1)	4 (3.4)	11 (3.2)
Patients with TEAE leading to discontinuation	0	1 (0.8)	1 (0.3)

TEAE, treatment-emergent adverse event.
† Occurring in ≥3% of patients in any treatment group.
‡ No serious TEAEs were considered by the investigators to be related to treatment.

Conclusions

- Among patients who applied ruxolitinib cream from Day 1 and did not achieve near-complete facial repigmentation (ie, <F-VASI90) at Week 52, improvements in F-VASI and T-VASI responses were observed with continued treatment through Week 104
  - 66.1% of patients achieved F-VASI75 and 63.8% achieved T-VASI50 with continued application of ruxolitinib cream
  - 33.9% attained F-VASI90 response after an additional 52 weeks of treatment
  - Achievement, maintenance, and improvement in response among patients who switched from vehicle to ruxolitinib cream after Week 24 were consistent with patients who applied ruxolitinib cream from Day 1
  - Response rates continued to increase throughout the study and did not plateau at Week 104
- Ruxolitinib cream was well tolerated, with no serious treatment-related AEs reported through 104 weeks