



Clinical trial results:

A Double-Blind, Vehicle-Controlled, Randomized Withdrawal and Treatment Extension Study to Assess the Long-Term Efficacy and Safety of Ruxolitinib Cream in Participants With Vitiligo

Summary

| | |
|--------------------------|------------------|
| EudraCT number | 2020-000987-53 |
| Trial protocol | DE NL FR PL BG |
| Global end of trial date | 14 November 2022 |

Results information

| | |
|--------------------------------|--------------|
| Result version number | v1 (current) |
| This version publication date | 28 May 2023 |
| First version publication date | 28 May 2023 |

Trial information

Trial identification

| | |
|-----------------------|----------------|
| Sponsor protocol code | INCB 18424-308 |
|-----------------------|----------------|

Additional study identifiers

| | |
|------------------------------------|---|
| ISRCTN number | - |
| ClinicalTrials.gov id (NCT number) | - |
| WHO universal trial number (UTN) | - |

Notes:

Sponsors

| | |
|------------------------------|--|
| Sponsor organisation name | Incyte Corporation |
| Sponsor organisation address | 1801 Augustine Cutoff Drive, Wilmington, United States, 19803 |
| Public contact | Study Director, Incyte Corporation, 1 8554633463, medinfo@incyte.com |
| Scientific contact | Study Director, Incyte Corporation, 1 8554633463, medinfo@incyte.com |

Notes:

Paediatric regulatory details

| | |
|--|---------------------|
| Is trial part of an agreed paediatric investigation plan (PIP) | Yes |
| EMA paediatric investigation plan number(s) | EMA-002618-PIP02-20 |
| Does article 45 of REGULATION (EC) No 1901/2006 apply to this trial? | No |
| Does article 46 of REGULATION (EC) No 1901/2006 apply to this trial? | Yes |

Notes:

Results analysis stage

| | |
|--|------------------|
| Analysis stage | Final |
| Date of interim/final analysis | 14 November 2022 |
| Is this the analysis of the primary completion data? | No |
| Global end of trial reached? | Yes |
| Global end of trial date | 14 November 2022 |
| Was the trial ended prematurely? | No |

Notes:

General information about the trial

Main objective of the trial:

This study was conducted to evaluate the duration of clinical response of ruxolitinib cream in participants with vitiligo.

Protection of trial subjects:

This study was to have been performed in accordance with ethical principles that have their origin in the Declaration of Helsinki and conducted in adherence to the study Protocol, applicable Good Clinical Practices, and applicable laws and country-specific regulations in which the study was being conducted.

Background therapy: -

Evidence for comparator: -

| | |
|---|-------------------|
| Actual start date of recruitment | 25 September 2020 |
| Long term follow-up planned | No |
| Independent data monitoring committee (IDMC) involvement? | No |

Notes:

Population of trial subjects

Subjects enrolled per country

| | |
|--------------------------------------|--------------------|
| Country: Number of subjects enrolled | Bulgaria: 30 |
| Country: Number of subjects enrolled | Canada: 47 |
| Country: Number of subjects enrolled | France: 24 |
| Country: Number of subjects enrolled | Germany: 15 |
| Country: Number of subjects enrolled | Netherlands: 8 |
| Country: Number of subjects enrolled | Poland: 89 |
| Country: Number of subjects enrolled | Spain: 7 |
| Country: Number of subjects enrolled | United States: 238 |
| Worldwide total number of subjects | 458 |
| EEA total number of subjects | 173 |

Notes:

Subjects enrolled per age group

| | |
|---|---|
| In utero | 0 |
| Preterm newborn - gestational age < 37 wk | 0 |
| Newborns (0-27 days) | 0 |
| Infants and toddlers (28 days-23 months) | 0 |

| | |
|---------------------------|-----|
| Children (2-11 years) | 0 |
| Adolescents (12-17 years) | 58 |
| Adults (18-64 years) | 368 |
| From 65 to 84 years | 32 |
| 85 years and over | 0 |

Subject disposition

Recruitment

Recruitment details:

This study was conducted at 87 study centers in North America and Europe.

Pre-assignment

Screening details:

This randomized withdrawal and treatment-extension study (extension of treatment received in 2 parent studies: NCT04052425 or NCT04057573) was comprised of a 52-week extension treatment period and a 4-week safety follow-up period, starting 4 weeks (30 days) after the last application of study treatment or the last study visit.

Period 1

| | |
|------------------------------|---|
| Period 1 title | Overall Study (overall period) |
| Is this the baseline period? | Yes |
| Allocation method | Randomised - controlled |
| Blinding used | Double blind |
| Roles blinded | Subject, Investigator, Monitor, Data analyst, Carer, Assessor |

Arms

| | |
|------------------------------|-----------------------------|
| Are arms mutually exclusive? | Yes |
| Arm title | Cohort A: Vehicle cream BID |

Arm description:

Participants who completed treatment and achieved $\geq 90\%$ improvement from Baseline in the Facial Vitiligo Area Scoring Index score (\geq F-VASI90) at Week 52 in either of 2 parent studies (NCT04052425 or NCT04057573). In this study, these participants were randomized to receive vehicle cream twice daily (BID) for 52 weeks. Participants who experienced relapse ($< 75\%$ improvement from Baseline in the F-VASI score [$<$ F-VASI75]) received open-label ruxolitinib 1.5% cream BID for the duration of the study.

| | |
|--|----------------------------|
| Arm type | Vehicle |
| Investigational medicinal product name | Vehicle cream |
| Investigational medicinal product code | |
| Other name | |
| Pharmaceutical forms | Cream |
| Routes of administration | Cutaneous use, Topical use |

Dosage and administration details:

Matching cream

| | |
|------------------|--------------------------------------|
| Arm title | Cohort A: Ruxolitinib 1.5% cream BID |
|------------------|--------------------------------------|

Arm description:

Participants who completed treatment and achieved \geq F-VASI90 at Week 52 in either of 2 parent studies (NCT04052425 or NCT04057573). In this study, these participants were randomized to receive ruxolitinib 1.5% cream BID for 52 weeks. Participants who experienced relapse ($<$ F-VASI75) received open-label ruxolitinib 1.5% cream BID for the duration of the study.

| | |
|--|--------------|
| Arm type | Experimental |
| Investigational medicinal product name | ruxolitinib |
| Investigational medicinal product code | INCB018424 |
| Other name | |
| Pharmaceutical forms | Cream |
| Routes of administration | Topical use |

Dosage and administration details:

1.5% weight/weight (W/W) BID

| | |
|------------------|---|
| Arm title | Cohort B: Vehicle cream BID to ruxolitinib 1.5% cream BID |
|------------------|---|

Arm description:

Participants who completed treatment (vehicle cream BID for 24 weeks and ruxolitinib 1.5% BID for 28 weeks) and did not achieve \geq F-VASI90 at Week 52 in either of 2 parent studies (NCT04052425 or NCT04057573). In this study, these participants applied ruxolitinib 1.5% cream BID for 52 weeks.

| | |
|--|--------------|
| Arm type | Experimental |
| Investigational medicinal product name | ruxolitinib |
| Investigational medicinal product code | INCB018424 |
| Other name | |
| Pharmaceutical forms | Cream |
| Routes of administration | Topical use |

Dosage and administration details:

1.5% weight/weight (W/W) BID

| | |
|------------------|--|
| Arm title | Cohort B: Ruxolitinib 1.5% cream to ruxolitinib 1.5% cream BID |
|------------------|--|

Arm description:

Participants who completed treatment (ruxolitinib 1.5% BID for 52 weeks) and did not achieve \geq F-VASI90 at Week 52 in either of 2 parent studies (NCT04052425 or NCT04057573). In this study, these participants applied ruxolitinib 1.5% cream BID for 52 weeks.

| | |
|--|--------------|
| Arm type | Experimental |
| Investigational medicinal product name | ruxolitinib |
| Investigational medicinal product code | INCB018424 |
| Other name | |
| Pharmaceutical forms | Cream |
| Routes of administration | Topical use |

Dosage and administration details:

1.5% weight/weight (W/W) BID

| Number of subjects in period 1 | Cohort A: Vehicle cream BID | Cohort A: Ruxolitinib 1.5% cream BID | Cohort B: Vehicle cream BID to ruxolitinib 1.5% cream BID |
|---|-----------------------------|--------------------------------------|---|
| | | | |
| Started | 58 | 58 | 118 |
| Completed | 41 | 50 | 92 |
| Not completed | 17 | 8 | 26 |
| Missed Safety Follow-up Visit | - | - | 1 |
| Consent withdrawn by subject | 14 | 5 | 18 |
| Physician decision | - | - | 1 |
| Discontinued due to COVID-19 Pandemic | - | - | 1 |
| Adverse event, non-fatal | - | - | - |
| Pregnancy | - | - | 2 |
| Not Compliant with Protocol-specific Visit Window | - | - | - |
| Personal Reasons | - | - | - |
| Lost to follow-up | 3 | 3 | 3 |
| Site Closed Due to Noncompliance | - | - | - |
| Lack of efficacy | - | - | - |
| Protocol deviation | - | - | - |

| Number of subjects in period 1 | Cohort B: Ruxolitinib 1.5% cream to ruxolitinib 1.5% cream BID |
|---|--|
| Started | 224 |
| Completed | 173 |
| Not completed | 51 |
| Missed Safety Follow-up Visit | - |
| Consent withdrawn by subject | 33 |
| Physician decision | 1 |
| Discontinued due to COVID-19 Pandemic | - |
| Adverse event, non-fatal | 1 |
| Pregnancy | 1 |
| Not Compliant with Protocol-specific Visit Window | 1 |
| Personal Reasons | 1 |
| Lost to follow-up | 10 |
| Site Closed Due to Noncompliance | 1 |
| Lack of efficacy | 1 |
| Protocol deviation | 1 |

Baseline characteristics

Reporting groups

| | |
|---|-----------------------------|
| Reporting group title | Cohort A: Vehicle cream BID |
| Reporting group description: | |
| Participants who completed treatment and achieved $\geq 90\%$ improvement from Baseline in the Facial Vitiligo Area Scoring Index score (\geq F-VASI90) at Week 52 in either of 2 parent studies (NCT04052425 or NCT04057573). In this study, these participants were randomized to receive vehicle cream twice daily (BID) for 52 weeks. Participants who experienced relapse ($< 75\%$ improvement from Baseline in the F-VASI score [$<$ F-VASI75]) received open-label ruxolitinib 1.5% cream BID for the duration of the study. | |

| | |
|---|--------------------------------------|
| Reporting group title | Cohort A: Ruxolitinib 1.5% cream BID |
| Reporting group description: | |
| Participants who completed treatment and achieved \geq F-VASI90 at Week 52 in either of 2 parent studies (NCT04052425 or NCT04057573). In this study, these participants were randomized to receive ruxolitinib 1.5% cream BID for 52 weeks. Participants who experienced relapse ($<$ F-VASI75) received open-label ruxolitinib 1.5% cream BID for the duration of the study. | |

| | |
|---|---|
| Reporting group title | Cohort B: Vehicle cream BID to ruxolitinib 1.5% cream BID |
| Reporting group description: | |
| Participants who completed treatment (vehicle cream BID for 24 weeks and ruxolitinib 1.5% BID for 28 weeks) and did not achieve \geq F-VASI90 at Week 52 in either of 2 parent studies (NCT04052425 or NCT04057573). In this study, these participants applied ruxolitinib 1.5% cream BID for 52 weeks. | |

| | |
|--|--|
| Reporting group title | Cohort B: Ruxolitinib 1.5% cream to ruxolitinib 1.5% cream BID |
| Reporting group description: | |
| Participants who completed treatment (ruxolitinib 1.5% BID for 52 weeks) and did not achieve \geq F-VASI90 at Week 52 in either of 2 parent studies (NCT04052425 or NCT04057573). In this study, these participants applied ruxolitinib 1.5% cream BID for 52 weeks. | |

| Reporting group values | Cohort A: Vehicle cream BID | Cohort A: Ruxolitinib 1.5% cream BID | Cohort B: Vehicle cream BID to ruxolitinib 1.5% cream BID |
|---|-----------------------------|--------------------------------------|---|
| Number of subjects | 58 | 58 | 118 |
| Age categorical | | | |
| Units: Subjects | | | |
| Adolescents (12-17 years) | 4 | 8 | 10 |
| Adults (18-64 years) | 53 | 46 | 99 |
| From 65-84 years | 1 | 4 | 9 |
| Age Continuous | | | |
| Units: years | | | |
| arithmetic mean | 39.3 | 42.9 | 39.7 |
| standard deviation | ± 12.49 | ± 15.95 | ± 14.62 |
| Sex: Female, Male | | | |
| Units: participants | | | |
| Female | 31 | 33 | 61 |
| Male | 27 | 25 | 57 |
| Race/Ethnicity, Customized | | | |
| Units: Subjects | | | |
| Asian | 4 | 3 | 3 |
| Black or African American | 5 | 4 | 3 |
| Native Hawaiian or Other Pacific Islander | 1 | 0 | 0 |
| Not Reported | 2 | 1 | 3 |

| | | | |
|--|----|----|-----|
| White | 42 | 48 | 107 |
| Captured as Latino in Database | 1 | 0 | 0 |
| Persian | 1 | 0 | 0 |
| Indo-Caribbean | 1 | 0 | 0 |
| Jordanian | 1 | 0 | 0 |
| Brazilian | 0 | 1 | 0 |
| Guyana | 0 | 1 | 0 |
| American Indian or Alaska Native | 0 | 0 | 0 |
| Cape Verdean | 0 | 0 | 1 |
| Dominican Republic | 0 | 0 | 1 |
| Captured as Hispanic or Latino in Database | 0 | 0 | 0 |
| Iranian | 0 | 0 | 0 |
| Indian | 0 | 0 | 0 |
| North African | 0 | 0 | 0 |
| Middle Eastern | 0 | 0 | 0 |
| Arab//North-African | 0 | 0 | 0 |
| White/Black/Asian | 0 | 0 | 0 |
| Mexican | 0 | 0 | 0 |
| Race/Ethnicity, Customized | | | |
| Units: Subjects | | | |
| Hispanic or Latino | 11 | 13 | 16 |
| Not Hispanic or Latino | 45 | 43 | 99 |
| Not Reported | 2 | 1 | 3 |
| Unknown | 0 | 0 | 0 |
| Captured as Other in Database | 0 | 1 | 0 |

| Reporting group values | Cohort B: Ruxolitinib 1.5% cream to ruxolitinib 1.5% cream BID | Total | |
|---|---|-------|--|
| Number of subjects | 224 | 458 | |
| Age categorical | | | |
| Units: Subjects | | | |
| Adolescents (12-17 years) | 36 | 58 | |
| Adults (18-64 years) | 170 | 368 | |
| From 65-84 years | 18 | 32 | |
| Age Continuous | | | |
| Units: years | | | |
| arithmetic mean | 39.3 | | |
| standard deviation | ± 16.45 | - | |
| Sex: Female, Male | | | |
| Units: participants | | | |
| Female | 129 | 254 | |
| Male | 95 | 204 | |
| Race/Ethnicity, Customized | | | |
| Units: Subjects | | | |
| Asian | 8 | 18 | |
| Black or African American | 11 | 23 | |
| Native Hawaiian or Other Pacific Islander | 1 | 2 | |
| Not Reported | 13 | 19 | |
| White | 180 | 377 | |

| | | | |
|--|-----|-----|--|
| Captured as Latino in Database | 0 | 1 | |
| Persian | 0 | 1 | |
| Indo-Caribbean | 0 | 1 | |
| Jordanian | 0 | 1 | |
| Brazilian | 0 | 1 | |
| Guyana | 0 | 1 | |
| American Indian or Alaska Native | 1 | 1 | |
| Cape Verdean | 1 | 2 | |
| Dominican Republic | 0 | 1 | |
| Captured as Hispanic or Latino in Database | 1 | 1 | |
| Iranian | 1 | 1 | |
| Indian | 1 | 1 | |
| North African | 1 | 1 | |
| Middle Eastern | 1 | 1 | |
| Arab//North-African | 1 | 1 | |
| White/Black/Asian | 1 | 1 | |
| Mexican | 2 | 2 | |
| Race/Ethnicity, Customized | | | |
| Units: Subjects | | | |
| Hispanic or Latino | 53 | 93 | |
| Not Hispanic or Latino | 157 | 344 | |
| Not Reported | 11 | 17 | |
| Unknown | 1 | 1 | |
| Captured as Other in Database | 2 | 3 | |

Subject analysis sets

| | |
|----------------------------|---|
| Subject analysis set title | Cohort A: Vehicle cream BID to ruxolitinib 1.5% cream BID |
| Subject analysis set type | Full analysis |

Subject analysis set description:

Participants who completed treatment and achieved $\geq 90\%$ improvement from Baseline in the Facial Vitiligo Area Scoring Index score (\geq F-VASI90) at Week 52 in either of 2 parent studies (NCT04052425 or NCT04057573). In this study, these participants were randomized to receive vehicle cream twice daily (BID) for 52 weeks. These participants experienced relapse ($<$ F-VASI75) and received open-label ruxolitinib 1.5% cream BID for the duration of the study.

| | |
|----------------------------|--------------------------------------|
| Subject analysis set title | Cohort B: Ruxolitinib 1.5% cream BID |
| Subject analysis set type | Full analysis |

Subject analysis set description:

Participants who completed treatment (vehicle cream BID for 24 weeks and ruxolitinib 1.5% BID for 28 weeks or ruxolitinib 1.5% BID for 52 weeks) and did not achieve \geq F-VASI90 at Week 52 in either of 2 parent studies (NCT04052425 or NCT04057573). In this study, these participants applied ruxolitinib 1.5% cream BID for 52 weeks and had at least 1 post-Baseline pharmacokinetic (PK) assessment.

| | |
|----------------------------|---|
| Subject analysis set title | Cohorts A and B: Ruxolitinib 1.5% cream BID |
| Subject analysis set type | Full analysis |

Subject analysis set description:

Participants who completed treatment and achieved \geq F-VASI90 at Week 52 in either of 2 parent studies (NCT04052425 or NCT04057573). Participants who completed treatment (vehicle cream BID for 24 weeks and ruxolitinib 1.5% BID for 28 weeks or ruxolitinib 1.5% BID for 52 weeks) and did not achieve \geq F-VASI90 at Week 52 in either of 2 parent studies (NCT04052425 or NCT04057573). In this study, these participants (58 from Cohort A; 289 from Cohort B) applied ruxolitinib 1.5% cream BID for 52 weeks And had at least 1 post-Baseline PK assessment.

| Reporting group values | Cohort A: Vehicle cream BID to ruxolitinib 1.5% cream BID | Cohort B: Ruxolitinib 1.5% cream BID | Cohorts A and B: Ruxolitinib 1.5% cream BID |
|--|---|--------------------------------------|---|
| Number of subjects | 23 | 293 | 347 |
| Age categorical Units: Subjects | | | |
| Adolescents (12-17 years) Adults (18-64 years) From 65-84 years | | | |
| Age Continuous Units: years arithmetic mean standard deviation | 3 ± | ± | ± |
| Sex: Female, Male Units: participants | | | |
| Female Male | | | |
| Race/Ethnicity, Customized Units: Subjects | | | |
| Asian Black or African American Native Hawaiian or Other Pacific Islander Not Reported White Captured as Latino in Database Persian Indo-Caribbean Jordanian Brazilian Guyana American Indian or Alaska Native Cape Verdean Dominican Republic Captured as Hispanic or Latino in Database Iranian Indian North African Middle Eastern Arab//North-African White/Black/Asian Mexican | | | |
| Race/Ethnicity, Customized Units: Subjects | | | |
| Hispanic or Latino Not Hispanic or Latino Not Reported Unknown Captured as Other in Database | | | |

End points

End points reporting groups

| | |
|-----------------------|-----------------------------|
| Reporting group title | Cohort A: Vehicle cream BID |
|-----------------------|-----------------------------|

Reporting group description:

Participants who completed treatment and achieved $\geq 90\%$ improvement from Baseline in the Facial Vitiligo Area Scoring Index score (\geq F-VASI90) at Week 52 in either of 2 parent studies (NCT04052425 or NCT04057573). In this study, these participants were randomized to receive vehicle cream twice daily (BID) for 52 weeks. Participants who experienced relapse ($< 75\%$ improvement from Baseline in the F-VASI score [$<$ F-VASI75]) received open-label ruxolitinib 1.5% cream BID for the duration of the study.

| | |
|-----------------------|--------------------------------------|
| Reporting group title | Cohort A: Ruxolitinib 1.5% cream BID |
|-----------------------|--------------------------------------|

Reporting group description:

Participants who completed treatment and achieved \geq F-VASI90 at Week 52 in either of 2 parent studies (NCT04052425 or NCT04057573). In this study, these participants were randomized to receive ruxolitinib 1.5% cream BID for 52 weeks. Participants who experienced relapse ($<$ F-VASI75) received open-label ruxolitinib 1.5% cream BID for the duration of the study.

| | |
|-----------------------|---|
| Reporting group title | Cohort B: Vehicle cream BID to ruxolitinib 1.5% cream BID |
|-----------------------|---|

Reporting group description:

Participants who completed treatment (vehicle cream BID for 24 weeks and ruxolitinib 1.5% BID for 28 weeks) and did not achieve \geq F-VASI90 at Week 52 in either of 2 parent studies (NCT04052425 or NCT04057573). In this study, these participants applied ruxolitinib 1.5% cream BID for 52 weeks.

| | |
|-----------------------|--|
| Reporting group title | Cohort B: Ruxolitinib 1.5% cream to ruxolitinib 1.5% cream BID |
|-----------------------|--|

Reporting group description:

Participants who completed treatment (ruxolitinib 1.5% BID for 52 weeks) and did not achieve \geq F-VASI90 at Week 52 in either of 2 parent studies (NCT04052425 or NCT04057573). In this study, these participants applied ruxolitinib 1.5% cream BID for 52 weeks.

| | |
|----------------------------|---|
| Subject analysis set title | Cohort A: Vehicle cream BID to ruxolitinib 1.5% cream BID |
|----------------------------|---|

| | |
|---------------------------|---------------|
| Subject analysis set type | Full analysis |
|---------------------------|---------------|

Subject analysis set description:

Participants who completed treatment and achieved $\geq 90\%$ improvement from Baseline in the Facial Vitiligo Area Scoring Index score (\geq F-VASI90) at Week 52 in either of 2 parent studies (NCT04052425 or NCT04057573). In this study, these participants were randomized to receive vehicle cream twice daily (BID) for 52 weeks. These participants experienced relapse ($<$ F-VASI75) and received open-label ruxolitinib 1.5% cream BID for the duration of the study.

| | |
|----------------------------|--------------------------------------|
| Subject analysis set title | Cohort B: Ruxolitinib 1.5% cream BID |
|----------------------------|--------------------------------------|

| | |
|---------------------------|---------------|
| Subject analysis set type | Full analysis |
|---------------------------|---------------|

Subject analysis set description:

Participants who completed treatment (vehicle cream BID for 24 weeks and ruxolitinib 1.5% BID for 28 weeks or ruxolitinib 1.5% BID for 52 weeks) and did not achieve \geq F-VASI90 at Week 52 in either of 2 parent studies (NCT04052425 or NCT04057573). In this study, these participants applied ruxolitinib 1.5% cream BID for 52 weeks and had at least 1 post-Baseline pharmacokinetic (PK) assessment.

| | |
|----------------------------|---|
| Subject analysis set title | Cohorts A and B: Ruxolitinib 1.5% cream BID |
|----------------------------|---|

| | |
|---------------------------|---------------|
| Subject analysis set type | Full analysis |
|---------------------------|---------------|

Subject analysis set description:

Participants who completed treatment and achieved \geq F-VASI90 at Week 52 in either of 2 parent studies (NCT04052425 or NCT04057573). Participants who completed treatment (vehicle cream BID for 24 weeks and ruxolitinib 1.5% BID for 28 weeks or ruxolitinib 1.5% BID for 52 weeks) and did not achieve \geq F-VASI90 at Week 52 in either of 2 parent studies (NCT04052425 or NCT04057573). In this study, these participants (58 from Cohort A; 289 from Cohort B) applied ruxolitinib 1.5% cream BID for 52 weeks And had at least 1 post-Baseline PK assessment.

Primary: Time to relapse (defined as $<$ F-VASI75)

| | |
|-----------------|---|
| End point title | Time to relapse (defined as $<$ F-VASI75) |
|-----------------|---|

End point description:

Relapse was defined as a loss of 75% improvement from Baseline in the Face Vitiligo Area Scoring Index score (F-VASI75) response, assessed as percentage improvement in the F-VASI score at Baseline (Day

1 of the parent study) to <75%. -9999, 9999=not estimable because there were too few events of loss of F-VASI75 response.

| | |
|----------------------------------|---------|
| End point type | Primary |
| End point timeframe: | |
| up to Week 52 of Extension Study | |

| End point values | Cohort A: Vehicle cream BID | Cohort A: Ruxolitinib 1.5% cream BID | Cohort B: Vehicle cream BID to ruxolitinib 1.5% cream BID | Cohort B: Ruxolitinib 1.5% cream to ruxolitinib 1.5% cream BID |
|----------------------------------|-----------------------------------|---|--|---|
| Subject group type | Reporting group | Reporting group | Reporting group | Reporting group |
| Number of subjects analysed | 56 | 55 | 0 ^[1] | 0 ^[2] |
| Units: days | | | | |
| median (confidence interval 95%) | 9999 (238.0 to 9999) | 9999 (-9999 to 9999) | (to) | (to) |

Notes:

[1] - Only participants in Cohort A were analyzed.

[2] - Only participants in Cohort A were analyzed.

Statistical analyses

| | |
|---|--|
| Statistical analysis title | Time to relapse treatment/vehicle |
| Statistical analysis description: | |
| Cox regression model stratified by stratification factor (treatment assignment in the parent studies) was conducted to compare the difference in hazard rate between treatment and vehicle. | |
| Comparison groups | Cohort A: Vehicle cream BID v Cohort A: Ruxolitinib 1.5% cream BID |
| Number of subjects included in analysis | 111 |
| Analysis specification | Pre-specified |
| Analysis type | superiority |
| P-value | = 0.0414 |
| Method | Logrank |
| Parameter estimate | Hazard ratio (HR) |
| Point estimate | 0.422 |
| Confidence interval | |
| level | 95 % |
| sides | 2-sided |
| lower limit | 0.18 |
| upper limit | 0.99 |

Secondary: Time to loss of adequate response

| | |
|---|-----------------------------------|
| End point title | Time to loss of adequate response |
| End point description: | |
| Loss of adequate response was defined as a loss of 90% improvement from Baseline in the F-VASI score (F-VASI90) response, assessed as percentage improvement in the F-VASI score at Baseline (Day 1 of the parent study) to <90%. -9999, 9999=not estimable because there were too few events of loss of F-VASI75 response. | |
| End point type | Secondary |

End point timeframe:
up to Week 52 of Extension Study

| End point values | Cohort A: Vehicle cream BID | Cohort A: Ruxolitinib 1.5% cream BID | Cohort B: Vehicle cream BID to ruxolitinib 1.5% cream BID | Cohort B: Ruxolitinib 1.5% cream to ruxolitinib 1.5% cream BID |
|----------------------------------|-----------------------------------|---|--|---|
| Subject group type | Reporting group | Reporting group | Reporting group | Reporting group |
| Number of subjects analysed | 56 | 55 | 0 ^[3] | 0 ^[4] |
| Units: days | | | | |
| median (confidence interval 95%) | 195.0 (113.0 to 372.0) | 9999 (-9999 to 9999) | (to) | (to) |

Notes:

[3] - Only participants in Cohort A were analyzed.

[4] - Only participants in Cohort A were analyzed.

Statistical analyses

| Statistical analysis title | Time to loss of response treatment/vehicle |
|--|--|
| Statistical analysis description: Cox regression model stratified by stratification factor (treatment assignment in the parent studies) was conducted to compare the difference in hazard rate between treatment and vehicle. | |
| Comparison groups | Cohort A: Vehicle cream BID v Cohort A: Ruxolitinib 1.5% cream BID |
| Number of subjects included in analysis | 111 |
| Analysis specification | Pre-specified |
| Analysis type | superiority |
| P-value | = 0.0003 |
| Method | Logrank |
| Parameter estimate | Hazard ratio (HR) |
| Point estimate | 0.316 |
| Confidence interval | |
| level | 95 % |
| sides | 2-sided |
| lower limit | 0.165 |
| upper limit | 0.606 |

Secondary: Percentage of participants achieving a $\geq 50\%$ improvement from Baseline in the Face Vitiligo Area Scoring Index (F-VASI50) score during the Extension Treatment Period

| | |
|-----------------|---|
| End point title | Percentage of participants achieving a $\geq 50\%$ improvement from Baseline in the Face Vitiligo Area Scoring Index (F-VASI50) score during the Extension Treatment Period |
|-----------------|---|

End point description:

An F-VASI50 responder achieved at least 50% improvement from Baseline in F-VASI, measured by the percentage of vitiligo involvement (percentage of body surface area [BSA]) and the degree of depigmentation: 0% (no depigmentation), 10% (only specks of depigmentation), 25% (pigmented area exceeded depigmented area), 50% (depigmented and pigmented area was equal), 75% (depigmented area exceeded pigmented area), 90% (specks of pigment), or 100% (no pigment). The percentage of

BSA (hand unit) vitiligo involvement was estimated to the nearest 0.1% by the Investigator using the Palmar Method. The Investigator used his/her hand to mimic the participant's hand size to evaluate the percentage of BSA vitiligo involvement. F-VASI was then derived by multiplying the values assessed for the vitiligo involvement by the percentage of affected skin for each site on the face and summing the values of all sites (possible range: 0-3; lower scores indicate increased improvement).

| | |
|--|-----------|
| End point type | Secondary |
| End point timeframe: | |
| up to Week 104 of Treatment Extension (Week 52 was the first visit of this Treatment Extension study.) | |

| End point values | Cohort A: Vehicle cream BID | Cohort A: Ruxolitinib 1.5% cream BID | Cohort B: Vehicle cream BID to ruxolitinib 1.5% cream BID | Cohort B: Ruxolitinib 1.5% cream to ruxolitinib 1.5% cream BID |
|-----------------------------------|-----------------------------------|---|--|---|
| Subject group type | Reporting group | Reporting group | Reporting group | Reporting group |
| Number of subjects analysed | 57 ^[5] | 57 ^[6] | 118 ^[7] | 222 ^[8] |
| Units: percentage of participants | | | | |
| number (confidence interval 95%) | | | | |
| Week 52, n=57, 57, 118, 221 | 98.2 (90.6 to 100.0) | 98.2 (90.6 to 100.0) | 45.8 (36.6 to 55.2) | 65.6 (58.9 to 71.9) |
| Week 56, n=56, 55, 108, 216 | 98.2 (90.4 to 100.0) | 96.4 (87.5 to 99.6) | 50.0 (40.2 to 59.8) | 69.4 (62.8 to 75.5) |
| Week 60, n=48, 52, 107, 211 | 97.9 (88.9 to 99.9) | 98.1 (89.7 to 100.0) | 54.2 (44.3 to 63.9) | 69.2 (62.5 to 75.4) |
| Week 64, n=43, 45, 108, 207 | 97.7 (87.7 to 99.9) | 100.0 (92.1 to 100.0) | 58.3 (48.5 to 67.7) | 72.0 (65.3 to 78.0) |
| Week 68, n=41, 45, 107, 210 | 95.1 (83.5 to 99.4) | 100.0 (92.1 to 100.0) | 63.6 (53.7 to 72.6) | 77.1 (70.9 to 82.6) |
| Week 80, n=30, 45, 99, 194 | 96.7 (82.8 to 99.9) | 100.0 (92.1 to 100.0) | 65.7 (55.4 to 74.9) | 78.9 (72.4 to 84.4) |
| Week 92, n=23, 43, 94, 179 | 100.0 (85.2 to 100.0) | 97.7 (87.7 to 99.9) | 66.0 (55.5 to 75.4) | 84.4 (78.2 to 89.3) |
| Week 104, n=23, 38, 93, 177 | 95.7 (78.1 to 99.9) | 100.0 (90.7 to 100.0) | 69.9 (59.5 to 79.0) | 86.4 (80.5 to 91.1) |

Notes:

[5] - Only participants with available data were analyzed.

[6] - Only participants with available data were analyzed.

[7] - Only participants with available data were analyzed.

[8] - Only participants with available data were analyzed.

Statistical analyses

No statistical analyses for this end point

Secondary: Percentage of participants achieving a $\geq 75\%$ improvement from Baseline in the F-VASI (F-VASI75) score during the Extension Treatment Period

| | |
|-----------------|---|
| End point title | Percentage of participants achieving a $\geq 75\%$ improvement from Baseline in the F-VASI (F-VASI75) score during the Extension Treatment Period |
|-----------------|---|

End point description:

An F-VASI75 responder achieved at least 75% improvement from Baseline in F-VASI, measured by the percentage of vitiligo involvement (percentage of BSA) and the degree of depigmentation: 0% (no depigmentation), 10% (only specks of depigmentation), 25% (pigmented area exceeded depigmented area), 50% (depigmented and pigmented area was equal), 75% (depigmented area exceeded pigmented area), 90% (specks of pigment), or 100% (no pigment). The percentage of BSA (hand unit)

vitiligo involvement was estimated to the nearest 0.1% by the Investigator using the Palmar Method. The Investigator used his/her hand to mimic the participant's hand size to evaluate the percentage of BSA vitiligo involvement. F-VASI was then derived by multiplying the values assessed for the vitiligo involvement by the percentage of affected skin for each site on the face and summing the values of all sites (possible range: 0-3; lower scores indicate increased improvement).

| | |
|--|-----------|
| End point type | Secondary |
| End point timeframe: | |
| up to Week 104 of Treatment Extension (Week 52 was the first visit of this Treatment Extension study.) | |

| End point values | Cohort A: Vehicle cream BID | Cohort A: Ruxolitinib 1.5% cream BID | Cohort B: Vehicle cream BID to ruxolitinib 1.5% cream BID | Cohort B: Ruxolitinib 1.5% cream to ruxolitinib 1.5% cream BID |
|-----------------------------------|-----------------------------------|---|--|---|
| Subject group type | Reporting group | Reporting group | Reporting group | Reporting group |
| Number of subjects analysed | 57 ^[9] | 57 ^[10] | 118 ^[11] | 222 ^[12] |
| Units: percentage of participants | | | | |
| number (confidence interval 95%) | | | | |
| Week 52, n=57, 57, 118, 221 | 98.2 (90.6 to 100.0) | 98.2 (90.6 to 100.0) | 16.1 (10.0 to 24.0) | 30.8 (24.8 to 37.3) |
| Week 56, n=56, 55, 108, 216 | 96.4 (87.7 to 99.6) | 96.4 (87.5 to 99.6) | 23.1 (15.6 to 32.2) | 34.7 (28.4 to 41.5) |
| Week 60, n=48, 52, 107, 211 | 95.8 (85.7 to 99.5) | 90.4 (79.0 to 96.8) | 29.0 (20.6 to 38.5) | 40.8 (34.1 to 47.7) |
| Week 64, n=43, 45, 108, 207 | 95.3 (84.2 to 99.4) | 97.8 (88.2 to 99.9) | 30.6 (22.1 to 40.2) | 43.5 (36.6 to 50.5) |
| Week 68, n=41, 45, 107, 210 | 87.8 (73.8 to 95.9) | 100.0 (92.1 to 100.0) | 34.6 (25.6 to 44.4) | 48.6 (41.6 to 55.5) |
| Week 80, n=30, 45, 99, 194 | 90.0 (73.5 to 97.9) | 100.0 (92.1 to 100.0) | 43.4 (33.5 to 53.8) | 54.6 (47.4 to 61.8) |
| Week 92, n=23, 43, 94, 179 | 100.0 (85.2 to 100.0) | 97.7 (87.7 to 99.9) | 47.9 (37.5 to 58.4) | 60.3 (52.8 to 67.6) |
| Week 104, n=23, 38, 93, 177 | 95.7 (78.1 to 99.9) | 97.4 (86.2 to 99.9) | 47.3 (36.9 to 57.9) | 66.1 (58.6 to 73.0) |

Notes:

[9] - Only participants with available data were analyzed.

[10] - Only participants with available data were analyzed.

[11] - Only participants with available data were analyzed.

[12] - Only participants with available data were analyzed.

Statistical analyses

No statistical analyses for this end point

Secondary: Percentage of participants achieving a $\geq 90\%$ improvement from Baseline in the F-VASI (F-VASI90) score during the Extension Treatment Period

| | |
|-----------------|---|
| End point title | Percentage of participants achieving a $\geq 90\%$ improvement from Baseline in the F-VASI (F-VASI90) score during the Extension Treatment Period |
|-----------------|---|

End point description:

An F-VASI90 responder achieved at least 90% improvement from Baseline in F-VASI, measured by the percentage of vitiligo involvement (percentage of BSA) and the degree of depigmentation: 0% (no depigmentation), 10% (only specks of depigmentation), 25% (pigmented area exceeded depigmented area), 50% (depigmented and pigmented area was equal), 75% (depigmented area exceeded pigmented area), 90% (specks of pigment), or 100% (no pigment). The percentage of BSA (hand unit)

vitiligo involvement was estimated to the nearest 0.1% by the Investigator using the Palmar Method. The Investigator used his/her hand to mimic the participant's hand size to evaluate the percentage of BSA vitiligo involvement. F-VASI was then derived by multiplying the values assessed for the vitiligo involvement by the percentage of affected skin for each site on the face and summing the values of all sites (possible range: 0-3; lower scores indicate increased improvement).

| | |
|--|-----------|
| End point type | Secondary |
| End point timeframe: | |
| up to Week 104 of Treatment Extension (Week 52 was the first visit of this Treatment Extension study.) | |

| End point values | Cohort A: Vehicle cream BID | Cohort A: Ruxolitinib 1.5% cream BID | Cohort B: Vehicle cream BID to ruxolitinib 1.5% cream BID | Cohort B: Ruxolitinib 1.5% cream to ruxolitinib 1.5% cream BID |
|-----------------------------------|-----------------------------------|---|--|---|
| Subject group type | Reporting group | Reporting group | Reporting group | Reporting group |
| Number of subjects analysed | 57 ^[13] | 57 ^[14] | 118 ^[15] | 222 ^[16] |
| Units: percentage of participants | | | | |
| number (confidence interval 95%) | | | | |
| Week 52, n=57, 57, 118, 221 | 98.2 (90.6 to 100.0) | 96.5 (87.9 to 99.6) | 0.0 (0.0 to 3.1) | 2.3 (0.7 to 5.2) |
| Week 56, n=56, 55, 108, 216 | 89.3 (78.1 to 96.0) | 94.5 (84.9 to 98.9) | 3.7 (1.0 to 9.2) | 8.3 (5.0 to 12.9) |
| Week 60, n=48, 52, 107, 211 | 83.3 (69.8 to 92.5) | 86.5 (74.2 to 94.4) | 8.4 (3.9 to 15.4) | 15.2 (10.6 to 20.7) |
| Week 64, n=43, 45, 108, 207 | 72.1 (56.3 to 84.7) | 93.3 (81.7 to 98.6) | 11.1 (5.9 to 18.6) | 15.5 (10.8 to 21.1) |
| Week 68, n=41, 45, 107, 210 | 68.3 (51.9 to 81.9) | 95.6 (84.9 to 99.5) | 12.1 (6.6 to 19.9) | 22.4 (16.9 to 28.6) |
| Week 80, n=30, 45, 99, 194 | 80.0 (61.4 to 92.3) | 97.8 (88.2 to 99.9) | 22.2 (14.5 to 31.7) | 30.4 (24.0 to 37.4) |
| Week 92, n=23, 43, 94, 179 | 78.3 (56.3 to 92.5) | 93.0 (80.9 to 98.5) | 24.5 (16.2 to 34.4) | 32.4 (25.6 to 39.8) |
| Week 104, n=23, 38, 93, 177 | 69.6 (47.1 to 86.8) | 92.1 (78.6 to 98.3) | 28.0 (19.1 to 38.2) | 33.9 (27.0 to 41.4) |

Notes:

[13] - Only participants with available data were analyzed.

[14] - Only participants with available data were analyzed.

[15] - Only participants with available data were analyzed.

[16] - Only participants with available data were analyzed.

Statistical analyses

No statistical analyses for this end point

Secondary: Mean F-VASI scores during the Extension Treatment Period

| | |
|-----------------|--|
| End point title | Mean F-VASI scores during the Extension Treatment Period |
|-----------------|--|

End point description:

F-VASI was measured by the percentage of vitiligo involvement (percentage of BSA) and the degree of depigmentation: 0% (no depigmentation), 10% (only specks of depigmentation), 25% (pigmented area exceeded depigmented area), 50% (depigmented and pigmented area was equal), 75% (depigmented area exceeded pigmented area), 90% (specks of pigment), or 100% (no pigment). The percentage of BSA (hand unit) vitiligo involvement was estimated to the nearest 0.1% by the Investigator using the Palmar Method. The Investigator used his/her hand to mimic the participant's hand size to evaluate the percentage of BSA vitiligo involvement. F-VASI was then derived by multiplying the values assessed for the vitiligo involvement by the percentage of affected skin for each site on the face and summing the

values of all sites (possible range: 0-3; lower scores indicate increased improvement).

| | |
|--|-----------|
| End point type | Secondary |
| End point timeframe: | |
| up to Week 104 of Treatment Extension (Week 52 was the first visit of this Treatment Extension study.) | |

| End point values | Cohort A: Vehicle cream BID | Cohort A: Ruxolitinib 1.5% cream BID | Cohort B: Vehicle cream BID to ruxolitinib 1.5% cream BID | Cohort B: Ruxolitinib 1.5% cream to ruxolitinib 1.5% cream BID |
|--------------------------------------|-----------------------------------|---|--|---|
| Subject group type | Reporting group | Reporting group | Reporting group | Reporting group |
| Number of subjects analysed | 57 ^[17] | 57 ^[18] | 118 ^[19] | 222 ^[20] |
| Units: scores on a scale | | | | |
| arithmetic mean (standard deviation) | | | | |
| Baseline, n=57, 57, 188, 222 | 0.86 (± 0.492) | 0.99 (± 0.644) | 0.88 (± 0.543) | 0.91 (± 0.550) |
| Week 52, n=57, 57, 118, 221 | 0.05 (± 0.110) | 0.07 (± 0.181) | 0.51 (± 0.459) | 0.39 (± 0.374) |
| Week 56, n=56, 55, 108, 216 | 0.05 (± 0.108) | 0.07 (± 0.186) | 0.50 (± 0.475) | 0.38 (± 0.372) |
| Week 60, n=48, 52, 107, 211 | 0.06 (± 0.145) | 0.09 (± 0.186) | 0.45 (± 0.450) | 0.35 (± 0.375) |
| Week 64, n=43, 45, 108, 207 | 0.08 (± 0.131) | 0.08 (± 0.230) | 0.43 (± 0.456) | 0.33 (± 0.350) |
| Week 68, n=43, 45, 108, 207 | 0.11 (± 0.199) | 0.04 (± 0.081) | 0.40 (± 0.430) | 0.31 (± 0.352) |
| Week 80, n=30, 45, 99, 194 | 0.05 (± 0.082) | 0.04 (± 0.108) | 0.36 (± 0.426) | 0.27 (± 0.345) |
| Week 92, n=23, 43, 94, 179 | 0.04 (± 0.055) | 0.04 (± 0.076) | 0.38 (± 0.471) | 0.23 (± 0.281) |
| Week 104, n=23, 38, 93, 177 | 0.06 (± 0.109) | 0.04 (± 0.083) | 0.37 (± 0.493) | 0.21 (± 0.280) |

Notes:

[17] - Only participants with available data were analyzed.

[18] - Only participants with available data were analyzed.

[19] - Only participants with available data were analyzed.

[20] - Only participants with available data were analyzed.

Statistical analyses

No statistical analyses for this end point

Secondary: Change from Baseline in F-VASI scores during the Extension Treatment Period

| | |
|-----------------|---|
| End point title | Change from Baseline in F-VASI scores during the Extension Treatment Period |
|-----------------|---|

End point description:

F-VASI was measured by the percentage of vitiligo involvement (percentage of BSA) and the degree of depigmentation: 0% (no depigmentation), 10% (only specks of depigmentation), 25% (pigmented area exceeded depigmented area), 50% (depigmented and pigmented area was equal), 75% (depigmented area exceeded pigmented area), 90% (specks of pigment), or 100% (no pigment). The percentage of BSA (hand unit) vitiligo involvement was estimated to the nearest 0.1% by the Investigator using the Palmar Method. The Investigator used his/her hand to mimic the participant's hand size to evaluate the percentage of BSA vitiligo involvement. F-VASI was then derived by multiplying the values assessed for the vitiligo involvement by the percentage of affected skin for each site on the face and summing the values of all sites (possible range: 0-3; lower scores indicate increased improvement). Change from Baseline was calculated as the post-Baseline value minus the Baseline value.

| | |
|----------------|-----------|
| End point type | Secondary |
|----------------|-----------|

End point timeframe:

Baseline; up to Week 104 of Treatment Extension (Week 52 was the first visit of this Treatment Extension study.)

| End point values | Cohort A: Vehicle cream BID | Cohort A: Ruxolitinib 1.5% cream BID | Cohort B: Vehicle cream BID to ruxolitinib 1.5% cream BID | Cohort B: Ruxolitinib 1.5% cream to ruxolitinib 1.5% cream BID |
|--------------------------------------|-----------------------------------|---|--|---|
| Subject group type | Reporting group | Reporting group | Reporting group | Reporting group |
| Number of subjects analysed | 57 ^[21] | 57 ^[22] | 118 ^[23] | 222 ^[24] |
| Units: scores on a scale | | | | |
| arithmetic mean (standard deviation) | | | | |
| Week 52, n=57, 57, 118, 221 | -0.81 (± 0.464) | -0.92 (± 0.591) | -0.37 (± 0.346) | -0.51 (± 0.448) |
| Week 56, n=56, 55, 108, 216 | -0.82 (± 0.492) | -0.90 (± 0.594) | -0.39 (± 0.370) | -0.54 (± 0.457) |
| Week 60, n=48, 52, 107, 211 | -0.84 (± 0.447) | -0.87 (± 0.570) | -0.43 (± 0.402) | -0.55 (± 0.457) |
| Week 64, n=43, 45, 108, 207 | -0.80 (± 0.442) | -0.87 (± 0.527) | -0.44 (± 0.423) | -0.58 (± 0.452) |
| Week 68, n=41, 45, 107, 210 | -0.73 (± 0.316) | -0.86 (± 0.518) | -0.46 (± 0.437) | -0.61 (± 0.476) |
| Week 80, n=30, 45, 99, 194 | -0.75 (± 0.360) | -0.86 (± 0.508) | -0.49 (± 0.471) | -0.64 (± 0.478) |
| Week 92, n=23, 43, 94, 179 | -0.76 (± 0.341) | -0.87 (± 0.548) | -0.48 (± 0.531) | -0.69 (± 0.488) |
| Week 104, n=23, 38, 93, 177 | -0.72 (± 0.380) | -0.92 (± 0.561) | -0.50 (± 0.557) | -0.68 (± 0.514) |

Notes:

[21] - Only participants with available data were analyzed.

[22] - Only participants with available data were analyzed.

[23] - Only participants with available data were analyzed.

[24] - Only participants with available data were analyzed.

Statistical analyses

No statistical analyses for this end point

Secondary: Percent change from Baseline in F-VASI scores during the Extension Treatment Period

| | |
|-----------------|---|
| End point title | Percent change from Baseline in F-VASI scores during the Extension Treatment Period |
|-----------------|---|

End point description:

F-VASI was measured by the percentage of vitiligo involvement (percentage of BSA) and the degree of depigmentation: 0% (no depigmentation), 10% (only specks of depigmentation), 25% (pigmented area exceeded depigmented area), 50% (depigmented and pigmented area was equal), 75% (depigmented area exceeded pigmented area), 90% (specks of pigment), or 100% (no pigment). The percentage of BSA (hand unit) vitiligo involvement was estimated to the nearest 0.1% by the Investigator using the Palmar Method. The Investigator used his/her hand to mimic the participant's hand size to evaluate the percentage of BSA vitiligo involvement. F-VASI was then derived by multiplying the values assessed for the vitiligo involvement by the percentage of affected skin for each site on the face and summing the values of all sites (possible range: 0-3; lower scores indicate increased improvement). Percentage change = ([post-BL value minus BL value]/BL value) X 100.

| | |
|----------------|-----------|
| End point type | Secondary |
|----------------|-----------|

End point timeframe:

Baseline (BL); up to Week 104 of Treatment Extension (Week 52 was the first visit of this Treatment Extension study.)

| End point values | Cohort A: Vehicle cream BID | Cohort A: Ruxolitinib 1.5% cream BID | Cohort B: Vehicle cream BID to ruxolitinib 1.5% cream BID | Cohort B: Ruxolitinib 1.5% cream to ruxolitinib 1.5% cream BID |
|--------------------------------------|-----------------------------------|---|--|---|
| Subject group type | Reporting group | Reporting group | Reporting group | Reporting group |
| Number of subjects analysed | 57 ^[25] | 57 ^[26] | 118 ^[27] | 222 ^[28] |
| Units: percentage change | | | | |
| arithmetic mean (standard deviation) | | | | |
| Week 52, n=57, 57, 118, 221 | -94.45 (± 14.069) | -94.50 (± 8.990) | -44.32 (± 27.775) | -54.72 (± 31.006) |
| Week 56, n=56, 55, 108, 216 | -93.79 (± 14.981) | -93.21 (± 12.573) | -46.19 (± 30.728) | -57.38 (± 31.256) |
| Week 60, n=48, 52, 107, 211 | -93.85 (± 9.087) | -92.04 (± 13.198) | -50.55 (± 30.537) | -60.17 (± 30.147) |
| Week 64, n=43, 45, 108, 207 | -89.64 (± 23.562) | -94.87 (± 8.079) | -52.32 (± 32.826) | -62.94 (± 27.427) |
| Week 68, n=41, 45, 107, 210 | -87.98 (± 19.644) | -96.25 (± 4.633) | -55.27 (± 32.433) | -65.55 (± 30.388) |
| Week 80, n=30, 45, 99, 194 | -93.24 (± 13.062) | -96.65 (± 4.683) | -58.60 (± 34.839) | -69.38 (± 29.091) |
| Week 92, n=23, 43, 94, 179 | -95.77 (± 5.586) | -94.95 (± 10.366) | -55.44 (± 49.855) | -73.77 (± 25.490) |
| Week 104, n=23, 38, 93, 177 | -90.53 (± 17.763) | -95.86 (± 7.213) | -56.53 (± 57.087) | -73.84 (± 31.780) |

Notes:

[25] - Only participants with available data were analyzed.

[26] - Only participants with available data were analyzed.

[27] - Only participants with available data were analyzed.

[28] - Only participants with available data were analyzed.

Statistical analyses

No statistical analyses for this end point

Secondary: Percentage of participants achieving a ≥50% improvement from Baseline in the Total Body Vitiligo Area Scoring Index (T-VASI50) score during the Extension Treatment Period

| | |
|-----------------|--|
| End point title | Percentage of participants achieving a ≥50% improvement from Baseline in the Total Body Vitiligo Area Scoring Index (T-VASI50) score during the Extension Treatment Period |
|-----------------|--|

End point description:

A T-VASI50 responder achieved at least 50% improvement from Baseline in T-VASI, calculated with contributions from 6 sites. The percentage of vitiligo involvement was estimated in hand units (percentage of BSA estimated to nearest 0.1%) by the Investigator using the Palmar Method. The Investigator used his/her hand to mimic the participant's hand size to evaluate percent BSA vitiligo involvement. The degree of depigmentation for each site was estimated to the nearest percentage: 0% (no depigmentation present), 10% (only specks of depigmentation present), 25% (pigmented area exceeded depigmented area), 50% (depigmented and pigmented area was equal), 75% (depigmented area exceeded pigmented area), 90% (specks of pigment present), 100% (no pigment present). T-VASI was then derived by multiplying the values assessed for the vitiligo involvement by the percentage of affected skin for each site and summing the values (range: 0-100; lower scores indicate increased improvement).

| | |
|----------------|-----------|
| End point type | Secondary |
|----------------|-----------|

End point timeframe:

up to Week 104 of Treatment Extension (Week 52 was the first visit of this Treatment Extension study.)

| End point values | Cohort A: Vehicle cream BID | Cohort A: Ruxolitinib 1.5% cream BID | Cohort B: Vehicle cream BID to ruxolitinib 1.5% cream BID | Cohort B: Ruxolitinib 1.5% cream to ruxolitinib 1.5% cream BID |
|-----------------------------------|-----------------------------------|---|--|---|
| Subject group type | Reporting group | Reporting group | Reporting group | Reporting group |
| Number of subjects analysed | 57 ^[29] | 57 ^[30] | 118 ^[31] | 222 ^[32] |
| Units: percentage of participants | | | | |
| number (confidence interval 95%) | | | | |
| Week 52, n=57, 57, 118, 221 | 80.7 (68.1 to 90.0) | 71.9 (58.5 to 83.0) | 16.9 (10.7 to 25.0) | 42.5 (35.9 to 49.3) |
| Week 56, n=56, 55, 108, 216 | 76.8 (63.6 to 87.0) | 72.7 (59.0 to 83.9) | 16.7 (10.2 to 25.1) | 45.8 (39.1 to 52.7) |
| Week 60, n=48, 52, 107, 211 | 83.3 (69.8 to 92.5) | 75.0 (61.1 to 86.0) | 19.6 (12.6 to 28.4) | 49.8 (42.8 to 56.7) |
| Week 64, n=43, 45, 108, 207 | 74.4 (58.8 to 86.5) | 82.2 (67.9 to 92.0) | 21.3 (14.0 to 30.2) | 50.7 (43.7 to 57.7) |
| Week 68, n=41, 45, 107, 210 | 70.7 (54.5 to 83.9) | 84.4 (70.5 to 93.5) | 29.9 (21.4 to 39.5) | 54.3 (47.3 to 61.2) |
| Week 80, n=30, 45, 99, 194 | 80.0 (61.4 to 92.3) | 86.7 (73.2 to 94.9) | 39.4 (29.7 to 49.7) | 57.7 (50.4 to 64.8) |
| Week 92, n=23, 43, 94, 179 | 73.9 (51.6 to 89.8) | 86.0 (72.1 to 94.7) | 48.9 (38.5 to 59.5) | 61.5 (53.9 to 68.6) |
| Week 104, n=23, 38, 93, 177 | 60.9 (38.5 to 80.3) | 89.5 (75.2 to 97.1) | 54.8 (44.2 to 65.2) | 63.8 (56.3 to 70.9) |

Notes:

[29] - Only participants with available data were analyzed.

[30] - Only participants with available data were analyzed.

[31] - Only participants with available data were analyzed.

[32] - Only participants with available data were analyzed.

Statistical analyses

No statistical analyses for this end point

Secondary: Percentage of participants achieving a $\geq 75\%$ improvement from Baseline in the T-VASI (T-VASI75) score during the Extension Treatment Period

| | |
|-----------------|---|
| End point title | Percentage of participants achieving a $\geq 75\%$ improvement from Baseline in the T-VASI (T-VASI75) score during the Extension Treatment Period |
|-----------------|---|

End point description:

A T-VASI75 responder achieved at least 75% improvement from Baseline in T-VASI, calculated with contributions from 6 sites. The percentage of vitiligo involvement was estimated in hand units (percentage of BSA estimated to nearest 0.1%) by the Investigator using the Palmar Method. The Investigator used his/her hand to mimic the participant's hand size to evaluate percent BSA vitiligo involvement. The degree of depigmentation for each site was estimated to the nearest percentage: 0% (no depigmentation present), 10% (only specks of depigmentation present), 25% (pigmented area exceeded depigmented area), 50% (depigmented and pigmented area was equal), 75% (depigmented area exceeded pigmented area), 90% (specks of pigment present), 100% (no pigment present). T-VASI was then derived by multiplying the values assessed for the vitiligo involvement by the percentage of affected skin for each site and summing the values (range: 0-100; lower scores indicate increased improvement).

| | |
|----------------|-----------|
| End point type | Secondary |
|----------------|-----------|

End point timeframe:

up to Week 104 of Treatment Extension (Week 52 was the first visit of this Treatment Extension study.)

| End point values | Cohort A: Vehicle cream BID | Cohort A: Ruxolitinib 1.5% cream BID | Cohort B: Vehicle cream BID to ruxolitinib 1.5% cream BID | Cohort B: Ruxolitinib 1.5% cream to ruxolitinib 1.5% cream BID |
|-----------------------------------|-----------------------------------|---|--|---|
| Subject group type | Reporting group | Reporting group | Reporting group | Reporting group |
| Number of subjects analysed | 57 ^[33] | 57 ^[34] | 118 ^[35] | 222 ^[36] |
| Units: percentage of participants | | | | |
| number (confidence interval 95%) | | | | |
| Week 52, n=57, 57, 118, 221 | 38.6 (26.0 to 52.4) | 42.1 (29.1 to 55.9) | 3.4 (0.9 to 8.5) | 12.2 (8.2 to 17.3) |
| Week 56, n=56, 55, 108, 216 | 42.9 (29.7 to 56.8) | 43.6 (30.3 to 57.7) | 4.6 (1.5 to 10.5) | 13.4 (9.2 to 18.7) |
| Week 60, n=48, 52, 107, 211 | 45.8 (31.4 to 60.8) | 38.5 (25.3 to 53.0) | 4.7 (1.5 to 10.6) | 14.2 (9.8 to 19.7) |
| Week 64, n=43, 45, 108, 207 | 39.5 (25.0 to 55.6) | 48.9 (33.7 to 64.2) | 3.7 (1.0 to 9.2) | 16.9 (12.1 to 22.7) |
| Week 68, n=41, 45, 107, 210 | 39.0 (24.2 to 55.5) | 42.2 (27.7 to 57.8) | 6.5 (2.7 to 13.0) | 22.4 (16.9 to 28.6) |
| Week 80, n=30, 45, 99, 194 | 53.3 (34.3 to 71.7) | 48.9 (33.7 to 64.2) | 10.1 (5.0 to 17.8) | 23.7 (17.9 to 30.3) |
| Week 92, n=23, 43, 94, 179 | 43.5 (23.2 to 65.5) | 53.5 (37.7 to 68.8) | 12.8 (6.8 to 21.2) | 29.1 (22.5 to 36.3) |
| Week 104, n=23, 38, 93, 177 | 39.1 (19.7 to 61.5) | 55.3 (38.3 to 71.4) | 18.3 (11.0 to 27.6) | 30.5 (23.8 to 37.9) |

Notes:

[33] - Only participants with available data were analyzed.

[34] - Only participants with available data were analyzed.

[35] - Only participants with available data were analyzed.

[36] - Only participants with available data were analyzed.

Statistical analyses

No statistical analyses for this end point

Secondary: Percentage of participants achieving a ≥90% improvement from Baseline in the T-VASI (T-VASI90) score during the Extension Treatment Period

| | |
|-----------------|--|
| End point title | Percentage of participants achieving a ≥90% improvement from Baseline in the T-VASI (T-VASI90) score during the Extension Treatment Period |
|-----------------|--|

End point description:

A T-VASI90 responder achieved at least 90% improvement from Baseline in T-VASI, calculated with contributions from 6 sites. The percentage of vitiligo involvement was estimated in hand units (percentage of BSA estimated to nearest 0.1%) by the Investigator using the Palmar Method. The Investigator used his/her hand to mimic the participant's hand size to evaluate percent BSA vitiligo involvement. The degree of depigmentation for each site was estimated to the nearest percentage: 0% (no depigmentation present), 10% (only specks of depigmentation present), 25% (pigmented area exceeded depigmented area), 50% (depigmented and pigmented area was equal), 75% (depigmented area exceeded pigmented area), 90% (specks of pigment present), 100% (no pigment present). T-VASI was then derived by multiplying the values assessed for the vitiligo involvement by the percentage of affected skin for each site and summing the values (range: 0-100; lower scores indicate increased improvement).

| | |
|----------------|-----------|
| End point type | Secondary |
|----------------|-----------|

End point timeframe:

up to Week 104 of Treatment Extension (Week 52 was the first visit of this Treatment Extension study.)

| End point values | Cohort A: Vehicle cream BID | Cohort A: Ruxolitinib 1.5% cream BID | Cohort B: Vehicle cream BID to ruxolitinib 1.5% cream BID | Cohort B: Ruxolitinib 1.5% cream to ruxolitinib 1.5% cream BID |
|-----------------------------------|-----------------------------------|---|--|---|
| Subject group type | Reporting group | Reporting group | Reporting group | Reporting group |
| Number of subjects analysed | 57 ^[37] | 57 ^[38] | 118 ^[39] | 222 ^[40] |
| Units: percentage of participants | | | | |
| number (confidence interval 95%) | | | | |
| Week 52, n=57, 57, 118, 221 | 12.3 (5.1 to 23.7) | 12.3 (5.1 to 23.7) | 0.0 (0.0 to 3.1) | 2.3 (0.7 to 5.2) |
| Week 56, n=56, 55, 108, 216 | 12.5 (5.2 to 24.1) | 14.5 (6.5 to 26.7) | 0.0 (0.0 to 3.4) | 3.2 (1.3 to 6.6) |
| Week 60, n=48, 52, 107, 211 | 16.7 (7.5 to 30.2) | 17.3 (8.2 to 30.3) | 0.0 (0.0 to 3.4) | 3.8 (1.7 to 7.3) |
| Week 64, n=43, 45, 108, 207 | 20.9 (10.0 to 36.0) | 20.0 (9.6 to 34.6) | 0.0 (0.0 to 3.4) | 2.9 (1.1 to 6.2) |
| Week 68, n=41, 45, 107, 210 | 22.0 (10.6 to 37.6) | 20.0 (9.6 to 34.6) | 0.9 (0.0 to 5.1) | 4.3 (2.0 to 8.0) |
| Week 80, n=30, 45, 99, 194 | 20.0 (7.7 to 38.6) | 20.0 (9.6 to 34.6) | 1.0 (0.0 to 5.5) | 6.7 (3.6 to 11.2) |
| Week 92, n=23, 43, 94, 179 | 21.7 (7.5 to 43.7) | 20.9 (10.0 to 36.0) | 2.1 (0.3 to 7.5) | 8.9 (5.2 to 14.1) |
| Week 104, n=23, 38, 93, 177 | 21.7 (7.5 to 43.7) | 23.7 (11.4 to 40.2) | 3.2 (0.7 to 9.1) | 9.6 (5.7 to 14.9) |

Notes:

[37] - Only participants with available data were analyzed.

[38] - Only participants with available data were analyzed.

[39] - Only participants with available data were analyzed.

[40] - Only participants with available data were analyzed.

Statistical analyses

No statistical analyses for this end point

Secondary: Mean T-VASI scores during the Extension Treatment Period

| | |
|-----------------|--|
| End point title | Mean T-VASI scores during the Extension Treatment Period |
|-----------------|--|

End point description:

T-VASI was calculated with contributions from 6 sites. The percentage of vitiligo involvement was estimated in hand units (percentage of BSA estimated to nearest 0.1%) by the Investigator using the Palmar Method. The Investigator used his/her hand to mimic the participant's hand size to evaluate percent BSA vitiligo involvement. The degree of depigmentation for each site was estimated to the nearest percentage: 0% (no depigmentation present), 10% (only specks of depigmentation present), 25% (pigmented area exceeded depigmented area), 50% (depigmented and pigmented area was equal), 75% (depigmented area exceeded pigmented area), 90% (specks of pigment present), 100% (no pigment present). T-VASI was then derived by multiplying the values assessed for the vitiligo involvement by the percentage of affected skin for each site and summing the values (range: 0-100; lower scores indicate increased improvement).

| | |
|----------------|-----------|
| End point type | Secondary |
|----------------|-----------|

End point timeframe:

up to Week 104 of Treatment Extension (Week 52 was the first visit of this Treatment Extension study.)

| End point values | Cohort A: Vehicle cream BID | Cohort A: Ruxolitinib 1.5% cream BID | Cohort B: Vehicle cream BID to ruxolitinib 1.5% cream BID | Cohort B: Ruxolitinib 1.5% cream to ruxolitinib 1.5% cream BID |
|--------------------------------------|-----------------------------------|---|--|---|
| Subject group type | Reporting group | Reporting group | Reporting group | Reporting group |
| Number of subjects analysed | 57 ^[41] | 57 ^[42] | 118 ^[43] | 222 ^[44] |
| Units: scores on a scale | | | | |
| arithmetic mean (standard deviation) | | | | |
| Baseline, n=57, 57, 118, 222 | 6.06 (± 2.056) | 6.27 (± 2.030) | 6.69 (± 2.150) | 6.74 (± 2.006) |
| Week 52, n=57, 57, 118, 221 | 2.38 (± 2.206) | 2.36 (± 1.805) | 5.25 (± 3.248) | 3.90 (± 2.132) |
| Week 56, n=56, 55, 108, 216 | 2.39 (± 2.253) | 2.31 (± 1.843) | 5.06 (± 3.151) | 3.80 (± 2.213) |
| Week 60, n=48, 52, 107, 211 | 2.21 (± 2.176) | 2.30 (± 1.841) | 4.86 (± 3.150) | 3.58 (± 2.122) |
| Week 64, n=43, 45, 108, 207 | 2.33 (± 2.210) | 1.94 (± 1.717) | 4.85 (± 3.317) | 3.57 (± 2.051) |
| Week 68, n=41, 45, 107, 210 | 2.45 (± 2.231) | 1.76 (± 1.378) | 4.51 (± 3.060) | 3.43 (± 2.160) |
| Week 80, n=30, 45, 99, 194 | 2.05 (± 2.233) | 1.73 (± 1.331) | 4.18 (± 3.036) | 3.18 (± 2.017) |
| Week 92, n=23, 43, 94, 179 | 2.17 (± 1.913) | 1.65 (± 1.244) | 4.12 (± 3.477) | 3.04 (± 2.128) |
| Week 104, n=23, 38, 93, 177 | 2.86 (± 3.269) | 1.54 (± 1.227) | 4.00 (± 4.208) | 2.92 (± 2.061) |

Notes:

[41] - Only participants with available data were analyzed.

[42] - Only participants with available data were analyzed.

[43] - Only participants with available data were analyzed.

[44] - Only participants with available data were analyzed.

Statistical analyses

No statistical analyses for this end point

Secondary: Change from Baseline in T-VASI scores during the Extension Treatment Period

| | |
|-----------------|---|
| End point title | Change from Baseline in T-VASI scores during the Extension Treatment Period |
|-----------------|---|

End point description:

T-VASI was calculated with contributions from 6 sites. The percentage of vitiligo involvement was estimated in hand units (percentage of BSA estimated to nearest 0.1%) by the Investigator using the Palmar Method. The Investigator used his/her hand to mimic the participant's hand size to evaluate percent BSA vitiligo involvement. The degree of depigmentation for each site was estimated to the nearest percentage: 0% (no depigmentation present), 10% (only specks of depigmentation present), 25% (pigmented area exceeded depigmented area), 50% (depigmented and pigmented area was equal), 75% (depigmented area exceeded pigmented area), 90% (specks of pigment present), 100% (no pigment present). T-VASI was then derived by multiplying the values assessed for the vitiligo involvement by the percentage of affected skin for each site and summing the values (range: 0-100; lower scores indicate increased improvement). Change from Baseline=post-Baseline value minus the Baseline value.

| | |
|----------------|-----------|
| End point type | Secondary |
|----------------|-----------|

End point timeframe:

Baseline; up to Week 104 of Treatment Extension (Week 52 was the first visit of this Treatment Extension study.)

| End point values | Cohort A: Vehicle cream BID | Cohort A: Ruxolitinib 1.5% cream BID | Cohort B: Vehicle cream BID to ruxolitinib 1.5% cream BID | Cohort B: Ruxolitinib 1.5% cream to ruxolitinib 1.5% cream BID |
|--------------------------------------|-----------------------------------|---|--|---|
| Subject group type | Reporting group | Reporting group | Reporting group | Reporting group |
| Number of subjects analysed | 57 ^[45] | 57 ^[46] | 118 ^[47] | 222 ^[48] |
| Units: scores on a scale | | | | |
| arithmetic mean (standard deviation) | | | | |
| Week 52, n=57, 57, 118, 221 | -3.68 (± 1.564) | -3.91 (± 1.526) | -1.45 (± 2.271) | -2.83 (± 1.938) |
| Week 56, n=56, 55, 108, 216 | -3.71 (± 1.615) | -3.91 (± 1.503) | -1.69 (± 2.217) | -2.97 (± 2.086) |
| Week 60, n=48, 52, 107, 211 | -3.79 (± 1.692) | -3.95 (± 1.585) | -1.83 (± 2.161) | -3.16 (± 2.027) |
| Week 64, n=43, 45, 108, 207 | -3.60 (± 2.033) | -4.20 (± 1.569) | -1.84 (± 2.375) | -3.21 (± 2.060) |
| Week 68, n=41, 45, 107, 210 | -3.44 (± 1.789) | -4.24 (± 1.534) | -2.21 (± 2.273) | -3.35 (± 2.143) |
| Week 80, n=30, 45, 99, 194 | -3.79 (± 1.663) | -4.26 (± 1.519) | -2.49 (± 2.175) | -3.55 (± 2.109) |
| Week 92, n=23, 43, 94, 179 | -3.85 (± 1.545) | -4.33 (± 1.673) | -2.61 (± 2.624) | -3.73 (± 2.246) |
| Week 104, n=23, 38, 93, 177 | -3.05 (± 2.156) | -4.48 (± 1.702) | -2.71 (± 3.479) | -3.84 (± 2.151) |

Notes:

[45] - Only participants with available data were analyzed.

[46] - Only participants with available data were analyzed.

[47] - Only participants with available data were analyzed.

[48] - Only participants with available data were analyzed.

Statistical analyses

No statistical analyses for this end point

Secondary: Percent change from Baseline in T-VASI scores during the Extension Treatment Period

| | |
|-----------------|---|
| End point title | Percent change from Baseline in T-VASI scores during the Extension Treatment Period |
|-----------------|---|

End point description:

T-VASI was calculated with contributions from 6 sites. The percentage of vitiligo involvement was estimated in hand units (percentage of BSA estimated to nearest 0.1%) by the Investigator using the Palmar Method. The Investigator used his/her hand to mimic the participant's hand size to evaluate percent BSA vitiligo involvement. The degree of depigmentation for each site was estimated to the nearest percentage: 0% (no depigmentation present), 10% (only specks of depigmentation present), 25% (pigmented area exceeded depigmented area), 50% (depigmented and pigmented area was equal), 75% (depigmented area exceeded pigmented area), 90% (specks of pigment present), 100% (no pigment present). T-VASI was then derived by multiplying the values assessed for the vitiligo involvement by the percentage of affected skin for each site and summing the values (range: 0-100; lower scores indicate increased improvement). Percentage change = ([post-BL value minus BL value]/BL value) X 100.

| | |
|----------------|-----------|
| End point type | Secondary |
|----------------|-----------|

End point timeframe:

Baseline; up to Week 104 of Treatment Extension (Week 52 was the first visit of this Treatment Extension study.)

| End point values | Cohort A: Vehicle cream BID | Cohort A: Ruxolitinib 1.5% cream BID | Cohort B: Vehicle cream BID to ruxolitinib 1.5% cream BID | Cohort B: Ruxolitinib 1.5% cream to ruxolitinib 1.5% cream BID |
|--------------------------------------|-----------------------------------|---|--|---|
| Subject group type | Reporting group | Reporting group | Reporting group | Reporting group |
| Number of subjects analysed | 57 ^[49] | 57 ^[50] | 118 ^[51] | 222 ^[52] |
| Units: percentage change | | | | |
| arithmetic mean (standard deviation) | | | | |
| Week 52, n=57, 57, 118, 221 | -65.01 (± 23.969) | -64.95 (± 21.547) | -24.75 (± 2.271) | -42.14 (± 25.783) |
| Week 56, n=56, 55, 108, 216 | -65.16 (± 24.916) | -65.69 (± 22.042) | -28.05 (± 30.487) | -43.91 (± 27.408) |
| Week 60, n=48, 52, 107, 211 | -66.83 (± 26.478) | -65.58 (± 22.910) | -31.01 (± 28.944) | -47.00 (± 26.089) |
| Week 64, n=43, 45, 108, 207 | -62.82 (± 33.328) | -70.44 (± 20.212) | -30.85 (± 32.898) | -46.96 (± 26.395) |
| Week 68, n=41, 45, 107, 210 | -61.40 (± 29.204) | -71.89 (± 17.942) | -35.53 (± 32.135) | -49.41 (± 27.629) |
| Week 80, n=30, 45, 99, 194 | -68.99 (± 26.870) | -72.21 (± 17.852) | -40.97 (± 30.033) | -52.48 (± 26.991) |
| Week 92, n=23, 43, 94, 179 | -66.95 (± 24.885) | -72.55 (± 19.152) | -43.34 (± 34.425) | -55.00 (± 28.995) |
| Week 104, n=23, 38, 93, 177 | -58.38 (± 35.533) | -74.66 (± 17.727) | -45.52 (± 41.968) | -56.96 (± 27.409) |

Notes:

[49] - Only participants with available data were analyzed.

[50] - Only participants with available data were analyzed.

[51] - Only participants with available data were analyzed.

[52] - Only participants with available data were analyzed.

Statistical analyses

No statistical analyses for this end point

Secondary: Mean Facial Body Surface Area (F-BSA) scores during the Extension Treatment Period

| | |
|-----------------|--|
| End point title | Mean Facial Body Surface Area (F-BSA) scores during the Extension Treatment Period |
|-----------------|--|

End point description:

F-BSA involvement was the proportion of the facial body surface area with vitiligo. The area "Face" was defined as including the area on the forehead to the original hairline, on the cheek to the jawline vertically to the jawline and laterally from the corner of the mouth to the tragus. The area "Face" did not include surface area of the lips, scalp, ears, or neck, but included the nose and eyelids. Body surface area assessment was performed by the Palmar Method. Body surface area was estimated to the nearest 0.1%. The approximate size of the participant's entire palmar surface (i.e., the palm plus 5 digits) was considered as 1% BSA, and the approximate size of the participant's thumb was considered as 0.1% BSA.

| | |
|----------------|-----------|
| End point type | Secondary |
|----------------|-----------|

End point timeframe:

up to Week 104 of Treatment Extension (Week 52 was the first visit of this Treatment Extension study.)

| End point values | Cohort A: Vehicle cream BID | Cohort A: Ruxolitinib 1.5% cream BID | Cohort B: Vehicle cream BID to ruxolitinib 1.5% cream BID | Cohort B: Ruxolitinib 1.5% cream to ruxolitinib 1.5% cream BID |
|--------------------------------------|-----------------------------------|---|--|---|
| Subject group type | Reporting group | Reporting group | Reporting group | Reporting group |
| Number of subjects analysed | 57 ^[53] | 57 ^[54] | 118 ^[55] | 222 ^[56] |
| Units: scores on a scale | | | | |
| arithmetic mean (standard deviation) | | | | |
| Baseline, n=57, 57, 118, 222 | 0.92 (± 0.498) | 1.10 (± 0.745) | 1.01 (± 0.632) | 1.02 (± 0.636) |
| Week 52, n=57, 57, 118, 221 | 0.18 (± 0.240) | 0.27 (± 0.401) | 0.81 (± 0.641) | 0.70 (± 0.568) |
| Week 56, n=56, 55, 108, 216 | 0.18 (± 0.198) | 0.27 (± 0.416) | 0.77 (± 0.638) | 0.67 (± 0.572) |
| Week 60, n=48, 51, 107, 211 | 0.20 (± 0.225) | 0.25 (± 0.309) | 0.73 (± 0.616) | 0.63 (± 0.557) |
| Week 64, n=43, 45, 108, 207 | 0.20 (± 0.206) | 0.20 (± 0.271) | 0.71 (± 0.607) | 0.61 (± 0.566) |
| Week 68, n=41, 45, 107, 210 | 0.22 (± 0.258) | 0.16 (± 0.183) | 0.67 (± 0.581) | 0.57 (± 0.548) |
| Week 80, n=30, 45, 99, 194 | 0.12 (± 0.138) | 0.15 (± 0.200) | 0.62 (± 0.565) | 0.55 (± 0.564) |
| Week 92, n=23, 43, 94, 179 | 0.10 (± 0.130) | 0.15 (± 0.180) | 0.64 (± 0.595) | 0.49 (± 0.527) |
| Week 104, n=23, 38, 93, 177 | 0.13 (± 0.154) | 0.15 (± 0.181) | 0.62 (± 0.602) | 0.47 (± 0.509) |

Notes:

[53] - Only participants with available data were analyzed.

[54] - Only participants with available data were analyzed.

[55] - Only participants with available data were analyzed.

[56] - Only participants with available data were analyzed.

Statistical analyses

No statistical analyses for this end point

Secondary: Change from Baseline in F-BSA scores during the Extension Treatment Period

| | |
|-----------------|--|
| End point title | Change from Baseline in F-BSA scores during the Extension Treatment Period |
|-----------------|--|

End point description:

F-BSA involvement was the proportion of the facial body surface area with vitiligo. The area "Face" was defined as including the area on the forehead to the original hairline, on the cheek to the jawline vertically to the jawline and laterally from the corner of the mouth to the tragus. The area "Face" did not include surface area of the lips, scalp, ears, or neck, but included the nose and eyelids. Body surface area assessment was performed by the Palmar Method. Body surface area was estimated to the nearest 0.1%. The approximate size of the participant's entire palmar surface (i.e., the palm plus 5 digits) was considered as 1% BSA, and the approximate size of the participant's thumb was considered as 0.1% BSA. Change from Baseline was calculated as the post-Baseline value minus the Baseline value.

| | |
|----------------|-----------|
| End point type | Secondary |
|----------------|-----------|

End point timeframe:

Baseline; up to Week 104 of Treatment Extension (Week 52 was the first visit of this Treatment Extension study.)

| End point values | Cohort A: Vehicle cream BID | Cohort A: Ruxolitinib 1.5% cream BID | Cohort B: Vehicle cream BID to ruxolitinib 1.5% cream BID | Cohort B: Ruxolitinib 1.5% cream to ruxolitinib 1.5% cream BID |
|--------------------------------------|-----------------------------------|---|--|---|
| Subject group type | Reporting group | Reporting group | Reporting group | Reporting group |
| Number of subjects analysed | 57 ^[57] | 57 ^[58] | 118 ^[59] | 222 ^[60] |
| Units: scores on a scale | | | | |
| arithmetic mean (standard deviation) | | | | |
| Week 52, n=57, 57, 118, 221 | -0.74 (± 0.402) | -0.83 (± 0.638) | -0.20 (± 0.313) | -0.31 (± 0.435) |
| Week 56, n=56, 55, 108, 216 | -0.76 (± 0.458) | -0.82 (± 0.672) | -0.25 (± 0.345) | -0.35 (± 0.433) |
| Week 60, n=48, 51, 107, 211 | -0.77 (± 0.441) | -0.80 (± 0.601) | -0.28 (± 0.364) | -0.38 (± 0.454) |
| Week 64, n=43, 45, 108, 207 | -0.74 (± 0.446) | -0.83 (± 0.625) | -0.29 (± 0.417) | -0.41 (± 0.457) |
| Week 68, n=41, 45, 107, 210 | -0.69 (± 0.345) | -0.86 (± 0.657) | -0.31 (± 0.425) | -0.45 (± 0.489) |
| Week 80, n=30, 45, 99, 194 | -0.74 (± 0.381) | -0.87 (± 0.637) | -0.37 (± 0.494) | -0.48 (± 0.521) |
| Week 92, n=23, 43, 94, 179 | -0.76 (± 0.374) | -0.87 (± 0.678) | -0.36 (± 0.558) | -0.54 (± 0.532) |
| Week 104, n=23, 38, 93, 177 | -0.70 (± 0.400) | -0.93 (± 0.716) | -0.39 (± 0.604) | -0.54 (± 0.548) |

Notes:

[57] - Only participants with available data were analyzed.

[58] - Only participants with available data were analyzed.

[59] - Only participants with available data were analyzed.

[60] - Only participants with available data were analyzed.

Statistical analyses

No statistical analyses for this end point

Secondary: Percent change from Baseline in F-BSA scores during the Extension Treatment Period

| | |
|-----------------|--|
| End point title | Percent change from Baseline in F-BSA scores during the Extension Treatment Period |
|-----------------|--|

End point description:

F-BSA involvement was the proportion of the facial body surface area with vitiligo. The area "Face" was defined as including the area on the forehead to the original hairline, on the cheek to the jawline vertically to the jawline and laterally from the corner of the mouth to the tragus. The area "Face" did not include surface area of the lips, scalp, ears, or neck, but included the nose and eyelids. Body surface area assessment was performed by the Palmar Method. Body surface area was estimated to the nearest 0.1%. The approximate size of the participant's entire palmar surface (i.e., the palm plus 5 digits) was considered as 1% BSA, and the approximate size of the participant's thumb was considered as 0.1% BSA. Percentage change = ([post-Baseline (BL) value minus BL value]/BL value) X 100.

| | |
|----------------|-----------|
| End point type | Secondary |
|----------------|-----------|

End point timeframe:

Baseline; up to Week 104 of Treatment Extension (Week 52 was the first visit of this Treatment Extension study.)

| End point values | Cohort A: Vehicle cream BID | Cohort A: Ruxolitinib 1.5% cream BID | Cohort B: Vehicle cream BID to ruxolitinib 1.5% cream BID | Cohort B: Ruxolitinib 1.5% cream to ruxolitinib 1.5% cream BID |
|--------------------------------------|-----------------------------------|---|--|---|
| Subject group type | Reporting group | Reporting group | Reporting group | Reporting group |
| Number of subjects analysed | 57 ^[61] | 57 ^[62] | 118 ^[63] | 222 ^[64] |
| Units: percentage change | | | | |
| arithmetic mean (standard deviation) | | | | |
| Week 52, n=57, 57, 118, 221 | -80.75 (± 18.726) | -76.75 (± 23.470) | -22.15 (± 28.268) | -29.06 (± 36.354) |
| Week 56, n=56, 55, 108, 216 | -80.23 (± 21.828) | -74.42 (± 35.048) | -26.20 (± 30.604) | -33.66 (± 33.944) |
| Week 60, n=48, 51, 107, 211 | -79.20 (± 19.463) | -77.67 (± 20.946) | -30.04 (± 30.751) | -36.86 (± 33.289) |
| Week 64, n=43, 45, 108, 207 | -75.92 (± 32.175) | -80.06 (± 22.142) | -30.67 (± 33.473) | -39.58 (± 31.992) |
| Week 68, n=41, 45, 107, 210 | -77.11 (± 23.565) | -83.14 (± 21.999) | -32.60 (± 34.575) | -43.61 (± 32.549) |
| Week 80, n=30, 45, 99, 194 | -84.55 (± 18.730) | -84.67 (± 22.014) | -36.79 (± 36.826) | -46.38 (± 34.407) |
| Week 92, n=23, 43, 94, 179 | -87.81 (± 15.738) | -83.24 (± 23.746) | -34.05 (± 50.762) | -51.96 (± 31.510) |
| Week 104, n=23, 38, 93, 177 | -82.87 (± 23.245) | -84.09 (± 22.920) | -35.82 (± 52.646) | -52.85 (± 32.983) |

Notes:

[61] - Only participants with available data were analyzed.

[62] - Only participants with available data were analyzed.

[63] - Only participants with available data were analyzed.

[64] - Only participants with available data were analyzed.

Statistical analyses

No statistical analyses for this end point

Secondary: Mean Total Body Surface Area (T-BSA) scores during the Extension Treatment Period

| | |
|-----------------|---|
| End point title | Mean Total Body Surface Area (T-BSA) scores during the Extension Treatment Period |
|-----------------|---|

End point description:

T-BSA involvement was the proportion of the body surface area with vitiligo. Body surface area assessment was performed by the Palmar Method. Body surface area was estimated to the nearest 0.1%. The approximate size of the participant's entire palmar surface (i.e., the palm plus 5 digits) was considered as 1% BSA, and the approximate size of the participant's thumb was considered as 0.1% BSA.

| | |
|----------------|-----------|
| End point type | Secondary |
|----------------|-----------|

End point timeframe:

up to Week 104 of Treatment Extension (Week 52 was the first visit of this Treatment Extension study.)

| End point values | Cohort A: Vehicle cream BID | Cohort A: Ruxolitinib 1.5% cream BID | Cohort B: Vehicle cream BID to ruxolitinib 1.5% cream BID | Cohort B: Ruxolitinib 1.5% cream to ruxolitinib 1.5% cream BID |
|--------------------------------------|-----------------------------------|---|--|---|
| Subject group type | Reporting group | Reporting group | Reporting group | Reporting group |
| Number of subjects analysed | 57 ^[65] | 57 ^[66] | 118 ^[67] | 222 ^[68] |
| Units: scores on a scale | | | | |
| arithmetic mean (standard deviation) | | | | |
| Baseline, n=57, 57, 118, 222 | 6.79 (± 2.157) | 6.85 (± 1.924) | 7.42 (± 2.064) | 7.49 (± 2.006) |
| Week 52, n=57, 57, 118, 221 | 4.09 (± 2.838) | 4.20 (± 2.640) | 6.95 (± 3.403) | 5.96 (± 2.352) |
| Week 56, n=56, 55, 108, 216 | 4.11 (± 2.880) | 4.13 (± 2.788) | 6.73 (± 3.281) | 5.81 (± 2.558) |
| Week 60, n=48, 51, 107, 211 | 3.77 (± 2.756) | 4.09 (± 2.833) | 6.61 (± 3.262) | 5.64 (± 2.479) |
| Week 64, n=43, 45, 108, 207 | 3.98 (± 3.087) | 3.49 (± 2.452) | 6.67 (± 3.448) | 5.64 (± 2.483) |
| Week 68, n=41, 45, 107, 210 | 3.92 (± 2.813) | 3.27 (± 2.310) | 6.46 (± 3.239) | 5.51 (± 2.682) |
| Week 80, n=30, 45, 99, 194 | 3.72 (± 3.151) | 3.18 (± 2.232) | 6.09 (± 3.313) | 5.27 (± 2.581) |
| Week 92, n=23, 43, 94, 179 | 3.94 (± 2.834) | 3.04 (± 2.194) | 5.96 (± 3.646) | 5.15 (± 2.741) |
| Week 104, n=23, 38, 93, 177 | 4.56 (± 4.156) | 2.93 (± 2.301) | 5.91 (± 4.475) | 5.07 (± 2.699) |

Notes:

[65] - Only participants with available data were analyzed.

[66] - Only participants with available data were analyzed.

[67] - Only participants with available data were analyzed.

[68] - Only participants with available data were analyzed.

Statistical analyses

No statistical analyses for this end point

Secondary: Change from Baseline in T-BSA scores during the Extension Treatment Period

| | |
|-----------------|--|
| End point title | Change from Baseline in T-BSA scores during the Extension Treatment Period |
|-----------------|--|

End point description:

T-BSA involvement was the proportion of the body surface area with vitiligo. Body surface area assessment was performed by the Palmar Method. Body surface area was estimated to the nearest 0.1%. The approximate size of the participant's entire palmar surface (i.e., the palm plus 5 digits) was considered as 1% BSA, and the approximate size of the participant's thumb was considered as 0.1% BSA. Change from Baseline was calculated as the post-Baseline value minus the Baseline value.

| | |
|----------------|-----------|
| End point type | Secondary |
|----------------|-----------|

End point timeframe:

Baseline; up to Week 104 of Treatment Extension (Week 52 was the first visit of this Treatment Extension study.)

| End point values | Cohort A: Vehicle cream BID | Cohort A: Ruxolitinib 1.5% cream BID | Cohort B: Vehicle cream BID to ruxolitinib 1.5% cream BID | Cohort B: Ruxolitinib 1.5% cream to ruxolitinib 1.5% cream BID |
|--------------------------------------|-----------------------------------|---|--|---|
| Subject group type | Reporting group | Reporting group | Reporting group | Reporting group |
| Number of subjects analysed | 57 ^[69] | 57 ^[70] | 118 ^[71] | 222 ^[72] |
| Units: scores on a scale | | | | |
| arithmetic mean (standard deviation) | | | | |

| | | | | |
|-----------------------------|-----------------|-----------------|-----------------|-----------------|
| Week 52, n=57, 57, 118, 221 | -2.70 (± 1.830) | -2.64 (± 1.906) | -0.47 (± 2.325) | -1.52 (± 1.711) |
| Week 56, n=56, 55, 108, 216 | -2.74 (± 1.918) | -2.70 (± 1.983) | -0.73 (± 2.238) | -1.71 (± 2.059) |
| Week 60, n=48, 51, 107, 211 | -2.95 (± 1.910) | -2.75 (± 2.195) | -0.80 (± 2.128) | -1.86 (± 1.978) |
| Week 64, n=43, 45, 108, 207 | -2.73 (± 2.528) | -3.22 (± 1.883) | -0.73 (± 2.432) | -1.90 (± 2.052) |
| Week 68, n=41, 45, 107, 210 | -2.73 (± 1.958) | -3.34 (± 1.922) | -0.98 (± 2.262) | -2.03 (± 2.180) |
| Week 80, n=30, 45, 99, 194 | -3.05 (± 2.083) | -3.42 (± 1.809) | -1.31 (± 2.341) | -2.21 (± 2.169) |
| Week 92, n=23, 43, 94, 179 | -3.03 (± 1.664) | -3.58 (± 1.938) | -1.49 (± 2.753) | -2.36 (± 2.380) |
| Week 104, n=23, 38, 93, 177 | -2.18 (± 2.723) | -3.72 (± 2.075) | -1.52 (± 3.672) | -2.42 (± 2.307) |

Notes:

[69] - Only participants with available data were analyzed.

[70] - Only participants with available data were analyzed.

[71] - Only participants with available data were analyzed.

[72] - Only participants with available data were analyzed.

Statistical analyses

No statistical analyses for this end point

Secondary: Percent change from Baseline in T-BSA scores during the Extension Treatment Period

| | |
|-----------------|--|
| End point title | Percent change from Baseline in T-BSA scores during the Extension Treatment Period |
|-----------------|--|

End point description:

T-BSA involvement was the proportion of the body surface area with vitiligo. Body surface area assessment was performed by the Palmar Method. Body surface area was estimated to the nearest 0.1%. The approximate size of the participant's entire palmar surface (i.e., the palm plus 5 digits) was considered as 1% BSA, and the approximate size of the participant's thumb was considered as 0.1% BSA. Percentage change = ([post-Baseline (BL) value minus BL value]/BL value) X 100.

| | |
|----------------|-----------|
| End point type | Secondary |
|----------------|-----------|

End point timeframe:

Baseline; up to Week 104 of Treatment Extension (Week 52 was the first visit of this Treatment Extension study.)

| End point values | Cohort A: Vehicle cream BID | Cohort A: Ruxolitinib 1.5% cream BID | Cohort B: Vehicle cream BID to ruxolitinib 1.5% cream BID | Cohort B: Ruxolitinib 1.5% cream to ruxolitinib 1.5% cream BID |
|--------------------------------------|-----------------------------------|---|--|---|
| Subject group type | Reporting group | Reporting group | Reporting group | Reporting group |
| Number of subjects analysed | 57 ^[73] | 57 ^[74] | 118 ^[75] | 222 ^[76] |
| Units: percentage change | | | | |
| arithmetic mean (standard deviation) | | | | |
| Week 52, n=57, 57, 118, 221 | -43.81 (± 27.916) | -41.50 (± 28.747) | -8.54 (± 28.806) | -20.57 (± 23.042) |
| Week 56, n=56, 55, 108, 216 | -44.22 (± 29.590) | -43.12 (± 29.489) | -11.96 (± 28.156) | -22.98 (± 26.032) |

| | | | | |
|-----------------------------|-------------------|-------------------|-------------------|-------------------|
| Week 60, n=48, 51, 107, 211 | -47.61 (± 29.237) | -43.09 (± 31.608) | -13.40 (± 26.410) | -25.12 (± 25.564) |
| Week 64, n=43, 45, 108, 207 | -43.29 (± 40.045) | -50.59 (± 27.166) | -12.00 (± 30.202) | -25.15 (± 26.860) |
| Week 68, n=41, 45, 107, 210 | -44.53 (± 30.086) | -52.51 (± 26.921) | -15.01 (± 29.093) | -27.59 (± 28.476) |
| Week 80, n=30, 45, 99, 194 | -50.51 (± 33.794) | -53.98 (± 25.682) | -20.22 (± 30.154) | -29.92 (± 27.996) |
| Week 92, n=23, 43, 94, 179 | -48.53 (± 29.457) | -55.56 (± 26.401) | -22.84 (± 33.708) | -31.98 (± 30.805) |
| Week 104, n=23, 38, 93, 177 | -40.84 (± 40.076) | -57.40 (± 27.898) | -24.26 (± 42.193) | -32.96 (± 29.907) |

Notes:

[73] - Only participants with available data were analyzed.

[74] - Only participants with available data were analyzed.

[75] - Only participants with available data were analyzed.

[76] - Only participants with available data were analyzed.

Statistical analyses

No statistical analyses for this end point

Secondary: Percentage of participants achieving a Vitiligo Noticeability Scale (VNS) score of 4 or 5 during the Extension Treatment Period

| | |
|-----------------|---|
| End point title | Percentage of participants achieving a Vitiligo Noticeability Scale (VNS) score of 4 or 5 during the Extension Treatment Period |
|-----------------|---|

End point description:

The VNS is a patient-reported measure of vitiligo treatment success that is rated on a 5-point scale. The Baseline facial photograph was shown to the participants for reference, and a mirror was provided for the participants to assess the vitiligo on their face. The participant was asked to respond to the following query: Compared with before treatment, how noticeable is the vitiligo now? Responses: (1) more noticeable, (2) as noticeable, (3) slightly less noticeable, (4) a lot less noticeable, and (5) no longer noticeable.

| | |
|----------------|-----------|
| End point type | Secondary |
|----------------|-----------|

End point timeframe:

Baseline; up to Week 104 of Treatment Extension (Week 52 was the first visit of this Treatment Extension study.)

| End point values | Cohort A: Vehicle cream BID | Cohort A: Ruxolitinib 1.5% cream BID | Cohort B: Vehicle cream BID to ruxolitinib 1.5% cream BID | Cohort B: Ruxolitinib 1.5% cream to ruxolitinib 1.5% cream BID |
|-----------------------------------|-----------------------------------|---|--|---|
| Subject group type | Reporting group | Reporting group | Reporting group | Reporting group |
| Number of subjects analysed | 57 ^[77] | 57 ^[78] | 118 ^[79] | 222 ^[80] |
| Units: percentage of participants | | | | |
| number (confidence interval 95%) | | | | |
| Week 52, n=57, 57, 118, 221 | 49.1 (35.6 to 62.7) | 42.1 (29.1 to 55.9) | 11.9 (6.6 to 19.1) | 35.3 (29.0 to 42.0) |
| Week 56, n=56, 55, 108, 216 | 48.2 (34.7 to 62.0) | 34.5 (22.2 to 48.6) | 20.4 (13.2 to 29.2) | 30.1 (24.1 to 36.7) |
| Week 60, n=49, 52, 107, 210 | 46.9 (32.5 to 61.7) | 28.8 (17.1 to 43.1) | 19.6 (12.6 to 28.4) | 27.6 (21.7 to 34.2) |

| | | | | |
|-----------------------------|---------------------|---------------------|---------------------|---------------------|
| Week 64, n=43, 45, 108, 206 | 53.5 (37.7 to 68.8) | 37.8 (23.8 to 53.5) | 23.1 (15.6 to 32.2) | 28.2 (22.1 to 34.8) |
| Week 68, n=41, 45, 107, 210 | 46.3 (30.7 to 62.6) | 46.7 (31.7 to 62.1) | 20.6 (13.4 to 29.5) | 33.8 (27.4 to 40.6) |
| Week 80, n=31, 45, 99, 195 | 38.7 (21.8 to 57.8) | 42.2 (27.7 to 57.8) | 28.3 (19.7 to 38.2) | 32.8 (26.3 to 39.9) |
| Week 92, n=23, 43, 95, 180 | 39.1 (19.7 to 61.5) | 37.2 (23.0 to 53.3) | 28.4 (19.6 to 38.6) | 41.1 (33.8 to 48.7) |
| Week 104, n=23, 38, 93, 178 | 56.5 (34.5 to 76.8) | 50.0 (33.4 to 66.6) | 30.1 (21.0 to 40.5) | 43.3 (35.9 to 50.9) |

Notes:

[77] - Only participants with available data were analyzed.

[78] - Only participants with available data were analyzed.

[79] - Only participants with available data were analyzed.

[80] - Only participants with available data were analyzed.

Statistical analyses

No statistical analyses for this end point

Secondary: Change from Week 52 in Dermatology Life Quality Index (DLQI) total score during the Extension Treatment Period

| | |
|-----------------|--|
| End point title | Change from Week 52 in Dermatology Life Quality Index (DLQI) total score during the Extension Treatment Period |
|-----------------|--|

End point description:

The DLQI is a simple, 10-question validated questionnaire to measure how much the skin problem has affected the participant over the previous 7 days. Participants age ≥ 16 years answered the questionnaire with: (1) very much; (2) a lot; (3) a little; or (4) not at all. The questionnaire was analyzed under 6 headings: Symptoms and feelings (Questions 1 and 2); Daily activities (Questions 3 and 4); Leisure (Questions 5 and 6); Work and school (Question 7); Personal relations (Questions 8 and 9); and Treatment (Question 10). The total score range from 10 to 40; higher scores indicate higher quality of life. Change from Week 52 was calculated as the post-Week 52 value minus the Week 52 value.

| | |
|----------------|-----------|
| End point type | Secondary |
|----------------|-----------|

End point timeframe:

Week 52; up to up to Week 104 of Extension Study (Week 52 was the first visit of this Treatment Extension study.)

| End point values | Cohort A: Vehicle cream BID | Cohort A: Ruxolitinib 1.5% cream BID | Cohort B: Vehicle cream BID to ruxolitinib 1.5% cream BID | Cohort B: Ruxolitinib 1.5% cream to ruxolitinib 1.5% cream BID |
|--|-----------------------------------|---|--|---|
| Subject group type | Reporting group | Reporting group | Reporting group | Reporting group |
| Number of subjects analysed | 54 ^[81] | 51 ^[82] | 114 ^[83] | 200 ^[84] |
| Units: scores on a scale | | | | |
| arithmetic mean (standard deviation) | | | | |
| Week 52, n=54, 51, 114, 200 | 2.87 (\pm 3.059) | 4.10 (\pm 4.784) | 3.69 (\pm 3.428) | 3.53 (\pm 4.150) |
| Change from Week 52 at Week 56, n=53, 49, 104, 194 | 0.11 (\pm 2.054) | -0.47 (\pm 1.757) | -0.05 (\pm 2.138) | -0.22 (\pm 2.483) |
| Change from Week 52 at Week 60, n=46, 46, 103, 188 | 0.37 (\pm 2.388) | 0.13 (\pm 2.227) | 0.28 (\pm 2.491) | 0.24 (\pm 2.929) |
| Change from Week 52 at Week 64, n=40, 40, 104, 184 | 0.35 (\pm 2.578) | 0.18 (\pm 2.630) | -0.06 (\pm 2.712) | -0.14 (\pm 2.350) |

| | | | | |
|--|-----------------|-----------------|-----------------|-----------------|
| Change from Week 52 at Week 68, n=38, 40, 103, 188 | -0.08 (± 2.508) | -0.15 (± 2.202) | -0.17 (± 2.501) | 0.08 (± 2.454) |
| Change from Week 52 at Week 80, n=29, 40, 95, 175 | 0.03 (± 3.006) | -0.23 (± 2.315) | -0.77 (± 2.871) | -0.13 (± 2.809) |
| Change from Week 52 at Week 92, n=22, 39, 91, 160 | 0.27 (± 2.354) | -0.23 (± 1.898) | -0.89 (± 2.383) | -0.22 (± 2.941) |
| Change from Week 52 at Week 104, n=21, 35, 89, 158 | 0.57 (± 2.135) | -0.40 (± 1.538) | -0.48 (± 2.277) | -0.06 (± 2.813) |

Notes:

[81] - Only participants with available data were analyzed.

[82] - Only participants with available data were analyzed.

[83] - Only participants with available data were analyzed.

[84] - Only participants with available data were analyzed.

Statistical analyses

No statistical analyses for this end point

Secondary: Change from Week 52 in Children's Dermatology Life Quality Index (CDLQI) total score during the Extension Treatment Period

| | |
|-----------------|--|
| End point title | Change from Week 52 in Children's Dermatology Life Quality Index (CDLQI) total score during the Extension Treatment Period |
|-----------------|--|

End point description:

The CDLQI is the youth/children's version of the DLQI. The DLQI is a simple, 10-question validated questionnaire to measure how much the skin problem has affected the participant over the previous 7 days. Participants age <16 years answered the questionnaire with: (1) very much; (2) a lot; (3) a little; or (4) not at all. The questionnaire was analyzed under 6 headings: Symptoms and feelings (Questions 1 and 2); Leisure (Questions 4, 5, and 6); School or holidays (Question 7); Personal relationships (Questions 3 and 8); Sleep (Question 9); and Treatment (Question 10). The total score ranges from 10 to 40; higher scores indicate higher quality of life. Change from Week 52 was calculated as the post-Week 52 value minus the Week 52 value. 9999=the mean and standard deviation cannot be calculated for a single participant.

| | |
|----------------|-----------|
| End point type | Secondary |
|----------------|-----------|

End point timeframe:

Week 52; up to Week 104 of Treatment Extension (Week 52 was the first visit of this Treatment Extension study.)

| End point values | Cohort A: Vehicle cream BID | Cohort A: Ruxolitinib 1.5% cream BID | Cohort B: Vehicle cream BID to ruxolitinib 1.5% cream BID | Cohort B: Ruxolitinib 1.5% cream to ruxolitinib 1.5% cream BID |
|---|-----------------------------------|---|--|---|
| Subject group type | Reporting group | Reporting group | Reporting group | Reporting group |
| Number of subjects analysed | 3 ^[85] | 6 ^[86] | 4 ^[87] | 22 ^[88] |
| Units: scores on a scale | | | | |
| arithmetic mean (standard deviation) | | | | |
| Week 52, n=3, 6, 4, 22 | 1.67 (± 2.887) | 1.00 (± 0.632) | 1.25 (± 1.893) | 2.32 (± 3.872) |
| Change from Week 52 at Week 56, n=3, 6, 4, 22 | -1.00 (± 2.646) | -0.17 (± 0.753) | 0.00 (± 0.816) | 0.36 (± 1.989) |
| Change from Week 52 at Week 60, n=3, 6, 4, 22 | 0.00 (± 0.000) | -0.50 (± 0.548) | -0.50 (± 0.577) | -0.41 (± 2.108) |
| Change from Week 52 at Week 64, n=3, 5, 4, 22 | -1.33 (± 2.309) | 0.00 (± 0.707) | -1.00 (± 1.414) | -0.36 (± 1.399) |

| | | | | |
|--|-----------------|-----------------|-----------------|-----------------|
| Change from Week 52 at Week 68, n=3, 5, 4, 22 | -0.67 (± 1.155) | 0.40 (± 1.140) | 0.50 (± 0.577) | -0.23 (± 1.541) |
| Change from Week 52 at Week 80, n=2, 5, 4, 20 | -2.00 (± 2.828) | -0.40 (± 0.548) | -0.75 (± 1.708) | 0.15 (± 1.814) |
| Change from Week 52 at Week 92, n=1, 4, 4, 20 | 9999 (± 9999) | 0.25 (± 1.258) | -0.25 (± 0.957) | 0.70 (± 3.614) |
| Change from Week 52 at Week 104, n=2, 3, 4, 20 | -0.50 (± 0.707) | -0.67 (± 1.155) | -1.25 (± 1.893) | -0.20 (± 4.086) |

Notes:

[85] - Only participants with available data were analyzed.

[86] - Only participants with available data were analyzed.

[87] - Only participants with available data were analyzed.

[88] - Only participants with available data were analyzed.

Statistical analyses

No statistical analyses for this end point

Secondary: Number of participants with any treatment-emergent adverse event (TEAE)

| | |
|-----------------|---|
| End point title | Number of participants with any treatment-emergent adverse event (TEAE) |
|-----------------|---|

End point description:

A TEAE was defined as any adverse event (AE) reported for the first time or the worsening of a pre-existing event after the first application of study drug in this study. An AE was defined as any untoward medical occurrence associated with the use of a drug in humans, whether or not considered drug-related. An AE could therefore have been any unfavorable and unintended sign (including an abnormal laboratory finding), symptom, or disease (new or exacerbated) temporally associated with the use of study treatment.

| | |
|----------------|-----------|
| End point type | Secondary |
|----------------|-----------|

End point timeframe:

up to approximately Week 108 (Week 52 was the first visit of this Treatment Extension study.)

| End point values | Cohort A: Vehicle cream BID | Cohort A: Ruxolitinib 1.5% cream BID | Cohort B: Vehicle cream BID to ruxolitinib 1.5% cream BID | Cohort B: Ruxolitinib 1.5% cream to ruxolitinib 1.5% cream BID |
|-----------------------------|-----------------------------------|---|--|---|
| Subject group type | Reporting group | Reporting group | Reporting group | Reporting group |
| Number of subjects analysed | 58 | 58 | 118 | 224 |
| Units: participants | 21 | 32 | 59 | 114 |

| End point values | Cohort A: Vehicle cream BID to ruxolitinib 1.5% cream BID | | | |
|-----------------------------|--|--|--|--|
| Subject group type | Subject analysis set | | | |
| Number of subjects analysed | 23 | | | |
| Units: participants | 3 | | | |

Statistical analyses

No statistical analyses for this end point

Secondary: Trough plasma concentrations of ruxolitinib at Week 80 and Week 104

| | |
|-----------------|---|
| End point title | Trough plasma concentrations of ruxolitinib at Week 80 and Week 104 ^[89] |
|-----------------|---|

End point description:

The steady-state plasma concentration was assessed. Pharmacokinetic blood samples could have been collected at any time prior to study drug application at the site at the Week 80 visit and at any time at the Week 104 (End of Trial) visit.

| | |
|----------------|-----------|
| End point type | Secondary |
|----------------|-----------|

End point timeframe:

Weeks 80 (predose); Week 104 (any time post-dose) (Week 52 was the first visit of this Treatment Extension study.)

Notes:

[89] - The end point is not reporting statistics for all the arms in the baseline period. It is expected all the baseline period arms will be reported on when providing values for an end point on the baseline period.

Justification: Statistical analysis was not conducted for this endpoint.

| End point values | Cohort A: Ruxolitinib 1.5% cream BID | Cohort B: Ruxolitinib 1.5% cream BID | Cohorts A and B: Ruxolitinib 1.5% cream BID | |
|--------------------------------------|---|---|--|--|
| Subject group type | Reporting group | Subject analysis set | Subject analysis set | |
| Number of subjects analysed | 54 ^[90] | 293 ^[91] | 347 ^[92] | |
| Units: nanomolar | | | | |
| arithmetic mean (standard deviation) | | | | |
| 12 to <18 years, n=8, 40, 48 | 7.28 (± 11.7) | 5.56 (± 10.5) | 5.85 (± 10.6) | |
| 18 to <65 years, n=43, 229, 272 | 15.1 (± 19.5) | 12.7 (± 17.2) | 13.1 (± 17.6) | |
| ≥65 years, n=3, 24, 27 | 13.0 (± 9.44) | 25.7 (± 20.9) | 24.3 (± 20.2) | |
| Overall, n=54, 293, 347 | 13.8 (± 18.1) | 12.8 (± 17.4) | 12.9 (± 17.5) | |

Notes:

[90] - Only participants with available data were analyzed.

[91] - Only participants with available data were analyzed.

[92] - Only participants with available data were analyzed.

Statistical analyses

No statistical analyses for this end point

Adverse events

Adverse events information

Timeframe for reporting adverse events:

up to approximately Week 108 (Week 52 was the first visit of this Treatment Extension study.)

Adverse event reporting additional description:

Treatment-emergent adverse events, defined as adverse events reported for the first time or the worsening of pre-existing events after the first application of study drug, have been reported.

| | |
|-----------------|------------|
| Assessment type | Systematic |
|-----------------|------------|

Dictionary used

| | |
|-----------------|--------|
| Dictionary name | MedDRA |
|-----------------|--------|

| | |
|--------------------|------|
| Dictionary version | 23.1 |
|--------------------|------|

Reporting groups

| | |
|-----------------------|-------------------|
| Reporting group title | Vehicle cream BID |
|-----------------------|-------------------|

Reporting group description:

Participants who completed treatment and achieved $\geq 90\%$ improvement from Baseline in the Facial Vitiligo Area Scoring Index score (\geq F-VASI90) at Week 52 in either of 2 parent studies (NCT04052425 or NCT04057573). In this study, these participants were randomized to receive vehicle cream twice daily (BID) for 52 weeks. Participants who experienced relapse ($< 75\%$ improvement from Baseline in the F-VASI score [$<$ F-VASI75]) received open-label ruxolitinib 1.5% cream BID for the duration of the study.

| | |
|-----------------------|-------|
| Reporting group title | Total |
|-----------------------|-------|

Reporting group description:

Total

| | |
|-----------------------|----------------------------|
| Reporting group title | Ruxolitinib 1.5% cream BID |
|-----------------------|----------------------------|

Reporting group description:

Participants who completed treatment and achieved \geq F-VASI90 at Week 52 in either of 2 parent studies (NCT04052425 or NCT04057573). In this study, these participants were randomized to receive ruxolitinib 1.5% cream BID for 52 weeks, or were randomized to receive vehicle cream BID but experienced relapse ($<$ F-VASI75) and received open-label ruxolitinib 1.5% cream BID for the duration of the study. Participants who completed treatment (vehicle cream BID for 24 weeks and ruxolitinib 1.5% BID for 28 weeks) and did not achieve \geq F-VASI90 at Week 52 in either of 2 parent studies (NCT04052425 or NCT04057573). In this study, these participants applied ruxolitinib 1.5% cream BID for 52 weeks. Participants who completed treatment (ruxolitinib 1.5% BID for 52 weeks) and did not achieve \geq F-VASI90 at Week 52 in either of 2 parent studies (NCT04052425 or NCT04057573). In this study, these participants applied ruxolitinib 1.5% cream BID for 52 weeks.

| Serious adverse events | Vehicle cream BID | Total | Ruxolitinib 1.5% cream BID |
|---|-------------------|------------------|----------------------------|
| Total subjects affected by serious adverse events | | | |
| subjects affected / exposed | 0 / 58 (0.00%) | 12 / 458 (2.62%) | 12 / 423 (2.84%) |
| number of deaths (all causes) | 0 | 0 | 0 |
| number of deaths resulting from adverse events | 0 | 0 | 0 |
| Neoplasms benign, malignant and unspecified (incl cysts and polyps) | | | |
| Uterine leiomyoma | | | |
| subjects affected / exposed | 0 / 58 (0.00%) | 1 / 458 (0.22%) | 1 / 423 (0.24%) |
| occurrences causally related to treatment / all | 0 / 0 | 0 / 1 | 0 / 1 |
| deaths causally related to treatment / all | 0 / 0 | 0 / 0 | 0 / 0 |

| | | | |
|---|----------------|-----------------|-----------------|
| Injury, poisoning and procedural complications | | | |
| Spinal fracture | | | |
| subjects affected / exposed | 0 / 58 (0.00%) | 1 / 458 (0.22%) | 1 / 423 (0.24%) |
| occurrences causally related to treatment / all | 0 / 0 | 0 / 1 | 0 / 1 |
| deaths causally related to treatment / all | 0 / 0 | 0 / 0 | 0 / 0 |
| Hip fracture | | | |
| subjects affected / exposed | 0 / 58 (0.00%) | 1 / 458 (0.22%) | 1 / 423 (0.24%) |
| occurrences causally related to treatment / all | 0 / 0 | 0 / 1 | 0 / 1 |
| deaths causally related to treatment / all | 0 / 0 | 0 / 0 | 0 / 0 |
| Cardiac disorders | | | |
| Angina pectoris | | | |
| subjects affected / exposed | 0 / 58 (0.00%) | 1 / 458 (0.22%) | 1 / 423 (0.24%) |
| occurrences causally related to treatment / all | 0 / 0 | 0 / 1 | 0 / 1 |
| deaths causally related to treatment / all | 0 / 0 | 0 / 0 | 0 / 0 |
| Ear and labyrinth disorders | | | |
| Otosclerosis | | | |
| subjects affected / exposed | 0 / 58 (0.00%) | 1 / 458 (0.22%) | 1 / 423 (0.24%) |
| occurrences causally related to treatment / all | 0 / 0 | 0 / 1 | 0 / 1 |
| deaths causally related to treatment / all | 0 / 0 | 0 / 0 | 0 / 0 |
| Reproductive system and breast disorders | | | |
| Pelvic prolapse | | | |
| subjects affected / exposed | 0 / 58 (0.00%) | 1 / 458 (0.22%) | 1 / 423 (0.24%) |
| occurrences causally related to treatment / all | 0 / 0 | 0 / 1 | 0 / 1 |
| deaths causally related to treatment / all | 0 / 0 | 0 / 0 | 0 / 0 |
| Rectocele | | | |
| subjects affected / exposed | 0 / 58 (0.00%) | 1 / 458 (0.22%) | 1 / 423 (0.24%) |
| occurrences causally related to treatment / all | 0 / 0 | 0 / 1 | 0 / 1 |
| deaths causally related to treatment / all | 0 / 0 | 0 / 0 | 0 / 0 |
| Uterine prolapse | | | |
| subjects affected / exposed | 0 / 58 (0.00%) | 1 / 458 (0.22%) | 1 / 423 (0.24%) |
| occurrences causally related to treatment / all | 0 / 0 | 0 / 1 | 0 / 1 |
| deaths causally related to treatment / all | 0 / 0 | 0 / 0 | 0 / 0 |
| Cystocele | | | |

| | | | |
|---|----------------|-----------------|-----------------|
| subjects affected / exposed | 0 / 58 (0.00%) | 1 / 458 (0.22%) | 1 / 423 (0.24%) |
| occurrences causally related to treatment / all | 0 / 0 | 0 / 1 | 0 / 1 |
| deaths causally related to treatment / all | 0 / 0 | 0 / 0 | 0 / 0 |
| Hepatobiliary disorders | | | |
| Cholelithiasis | | | |
| subjects affected / exposed | 0 / 58 (0.00%) | 1 / 458 (0.22%) | 1 / 423 (0.24%) |
| occurrences causally related to treatment / all | 0 / 0 | 0 / 1 | 0 / 1 |
| deaths causally related to treatment / all | 0 / 0 | 0 / 0 | 0 / 0 |
| Respiratory, thoracic and mediastinal disorders | | | |
| Acute respiratory failure | | | |
| subjects affected / exposed | 0 / 58 (0.00%) | 1 / 458 (0.22%) | 1 / 423 (0.24%) |
| occurrences causally related to treatment / all | 0 / 0 | 0 / 1 | 0 / 1 |
| deaths causally related to treatment / all | 0 / 0 | 0 / 0 | 0 / 0 |
| Psychiatric disorders | | | |
| Bipolar I disorder | | | |
| subjects affected / exposed | 0 / 58 (0.00%) | 1 / 458 (0.22%) | 1 / 423 (0.24%) |
| occurrences causally related to treatment / all | 0 / 0 | 0 / 1 | 0 / 1 |
| deaths causally related to treatment / all | 0 / 0 | 0 / 0 | 0 / 0 |
| Mental status changes | | | |
| subjects affected / exposed | 0 / 58 (0.00%) | 1 / 458 (0.22%) | 1 / 423 (0.24%) |
| occurrences causally related to treatment / all | 0 / 0 | 0 / 1 | 0 / 1 |
| deaths causally related to treatment / all | 0 / 0 | 0 / 0 | 0 / 0 |
| Musculoskeletal and connective tissue disorders | | | |
| Spinal osteoarthritis | | | |
| subjects affected / exposed | 0 / 58 (0.00%) | 1 / 458 (0.22%) | 1 / 423 (0.24%) |
| occurrences causally related to treatment / all | 0 / 0 | 0 / 2 | 0 / 2 |
| deaths causally related to treatment / all | 0 / 0 | 0 / 0 | 0 / 0 |
| Intervertebral disc disorder | | | |
| subjects affected / exposed | 0 / 58 (0.00%) | 1 / 458 (0.22%) | 1 / 423 (0.24%) |
| occurrences causally related to treatment / all | 0 / 0 | 0 / 1 | 0 / 1 |
| deaths causally related to treatment / all | 0 / 0 | 0 / 0 | 0 / 0 |
| Infections and infestations | | | |
| COVID-19 pneumonia | | | |

| | | | |
|---|----------------|-----------------|-----------------|
| subjects affected / exposed | 0 / 58 (0.00%) | 1 / 458 (0.22%) | 1 / 423 (0.24%) |
| occurrences causally related to treatment / all | 0 / 0 | 0 / 1 | 0 / 1 |
| deaths causally related to treatment / all | 0 / 0 | 0 / 0 | 0 / 0 |

Frequency threshold for reporting non-serious adverse events: 5 %

| Non-serious adverse events | Vehicle cream BID | Total | Ruxolitinib 1.5% cream BID |
|---|-------------------|-------------------|----------------------------|
| Total subjects affected by non-serious adverse events | | | |
| subjects affected / exposed | 6 / 58 (10.34%) | 61 / 458 (13.32%) | 55 / 423 (13.00%) |
| Infections and infestations | | | |
| COVID-19 | | | |
| subjects affected / exposed | 6 / 58 (10.34%) | 61 / 458 (13.32%) | 55 / 423 (13.00%) |
| occurrences (all) | 6 | 63 | 57 |

More information

Substantial protocol amendments (globally)

Were there any global substantial amendments to the protocol? Yes

| Date | Amendment |
|-------------------|--|
| 28 September 2020 | The primary purpose of this amendment was to accommodate the European Medicines Agency Pediatric Committee recommendation regarding the Dermatology Life Quality Index/Children's Dermatology Life Quality Index endpoint and to align the pharmacokinetic endpoints with those of parent studies INCB 18424-306 and INCB 18424-307. |
| 10 November 2020 | The main purpose of this amendment was to add language to the Protocol to inform sites of alternative strategies to guarantee continuity of the clinical trial conduct and oversight in response to the coronavirus disease 2019 (COVID-19) pandemic. |

Notes:

Interruptions (globally)

Were there any global interruptions to the trial? No

Limitations and caveats

Limitations of the trial such as small numbers of subjects analysed or technical problems leading to unreliable data.

Data from participants at a single site (N = 2) were removed from all efficacy analyses performed on the ITT-Ext Population and FAS Cohort A owing to noncompliance with the Protocol in the parent study resulting in serious concerns with data quality.

Notes: