



Clinical trial results:

Abrocitinib Expanded Access Protocol for the Treatment of Adolescents and Adults With Moderate to Severe Atopic Dermatitis

Summary

| | |
|--------------------------|-------------------|
| EudraCT number | 2020-003610-12 |
| Trial protocol | NL AT ES GR BE |
| Global end of trial date | 02 September 2024 |

Results information

| | |
|--------------------------------|---------------|
| Result version number | v1 (current) |
| This version publication date | 09 March 2025 |
| First version publication date | 09 March 2025 |

Trial information

Trial identification

| | |
|-----------------------|----------|
| Sponsor protocol code | B7451064 |
|-----------------------|----------|

Additional study identifiers

| | |
|------------------------------------|-------------|
| ISRCTN number | - |
| ClinicalTrials.gov id (NCT number) | NCT04564755 |
| WHO universal trial number (UTN) | - |

Notes:

Sponsors

| | |
|------------------------------|--|
| Sponsor organisation name | Pfizer Inc |
| Sponsor organisation address | 66 Hudson Boulevard East, New York, United States, NY 10017 |
| Public contact | Pfizer ClinicalTrials.gov Call Center, Pfizer Inc, 001 8007181021, ClinicalTrials.gov_Inquiries@pfizer.com |
| Scientific contact | Pfizer ClinicalTrials.gov Call Center, Pfizer Inc, 001 8007181021, ClinicalTrials.gov_Inquiries@pfizer.com |

Notes:

Paediatric regulatory details

| | |
|--|-----|
| Is trial part of an agreed paediatric investigation plan (PIP) | No |
| 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 | 25 September 2024 |
| Is this the analysis of the primary completion data? | Yes |
| Primary completion date | 02 August 2024 |
| Global end of trial reached? | Yes |
| Global end of trial date | 02 September 2024 |
| Was the trial ended prematurely? | Yes |

Notes:

General information about the trial

Main objective of the trial:

To provide access to Abrocitinib to adolescent and adult participants with or without background topical therapy who had inadequate treatment options due to inadequate response or intolerance to available approved medicated topical and systemic therapies, underlying conditions that preclude use of available approved medicated topical and systemic therapies, or lack of availability or access to approved medicated topical and systemic therapies and need Abrocitinib as a possible treatment regimen for moderate to severe atopic dermatitis (AD).

Protection of trial subjects:

The study was in compliance with the ethical principles derived from the Declaration of Helsinki and in compliance with all International Council for Harmonisation (ICH) Good Clinical Practice (GCP) Guidelines. All the local regulatory requirements pertinent to safety of trials participants were followed.

Background therapy: -

Evidence for comparator: -

| | |
|---|------------------|
| Actual start date of recruitment | 18 November 2020 |
| Long term follow-up planned | No |
| Independent data monitoring committee (IDMC) involvement? | Yes |

Notes:

Population of trial subjects

Subjects enrolled per country

| | |
|--------------------------------------|-----------------------|
| Country: Number of subjects enrolled | Australia: 19 |
| Country: Number of subjects enrolled | Austria: 10 |
| Country: Number of subjects enrolled | Belgium: 16 |
| Country: Number of subjects enrolled | Canada: 25 |
| Country: Number of subjects enrolled | Netherlands: 1 |
| Country: Number of subjects enrolled | Russian Federation: 1 |
| Country: Number of subjects enrolled | Spain: 21 |
| Country: Number of subjects enrolled | Switzerland: 12 |
| Country: Number of subjects enrolled | Taiwan: 16 |
| Country: Number of subjects enrolled | United States: 191 |
| Worldwide total number of subjects | 312 |
| EEA total number of subjects | 48 |

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) | 33 |
| Adults (18-64 years) | 245 |
| From 65 to 84 years | 34 |
| 85 years and over | 0 |

Subject disposition

Recruitment

Recruitment details: -

Pre-assignment

Screening details:

A total of 316 participants were enrolled at 44 centers in 10 countries/regions.

Period 1

| | |
|------------------------------|---------------------------------|
| Period 1 title | Overall Period (overall period) |
| Is this the baseline period? | Yes |
| Allocation method | Not applicable |
| Blinding used | Not blinded |

Arms

| | |
|------------------|-------------|
| Arm title | Abrocitinib |
|------------------|-------------|

Arm description:

Participants received abrocitinib 100milligrams (mg) or 200 mg tablet orally once a day (QD)

| | |
|--|--------------|
| Arm type | Experimental |
| Investigational medicinal product name | Abrocitinib |
| Investigational medicinal product code | |
| Other name | |
| Pharmaceutical forms | Tablet |
| Routes of administration | Oral use |

Dosage and administration details:

Participants received Abrocitinib 200 mg tablet orally every day (QD)

| | |
|--|-------------|
| Investigational medicinal product name | Abrocitinib |
| Investigational medicinal product code | |
| Other name | |
| Pharmaceutical forms | Tablet |
| Routes of administration | Oral use |

Dosage and administration details:

Participants received Abrocitinib 100 mg tablet orally every day (QD)

| Number of subjects in period 1 | Abrocitinib |
|---|-------------|
| Started | 312 |
| Completed | 184 |
| Not completed | 128 |
| Adverse event, serious fatal | 2 |
| Physician decision | 1 |
| Consent withdrawn by subject | 35 |
| Other, unspecified | 1 |
| Pregnancy | 1 |
| Medication error without associated adverse event | 1 |

| | |
|-------------------------------|----|
| Study terminated by sponsor | 17 |
| Other, Adverse event | 29 |
| Lost to follow-up | 23 |
| Withdrawal by parent/guardian | 1 |
| Lack of efficacy | 12 |
| Protocol deviation | 5 |

Baseline characteristics

Reporting groups

| | |
|--|----------------|
| Reporting group title | Overall Period |
| Reporting group description: | |
| Participants received abrocitinib 100 mg or 200 mg tablet orally once a day (QD) | |

| Reporting group values | Overall Period | Total | |
|--|----------------|-------|--|
| Number of subjects | 312 | 312 | |
| Age categorical | | | |
| Units: Subjects | | | |
| In Utero | 0 | 0 | |
| Preterm newborn infants (gestational age < 37 wks) | 0 | 0 | |
| Newborns (0-27 days) | 0 | 0 | |
| Infants and toddlers (28 days - 23 months) | 0 | 0 | |
| Children (2 - 11 years) | 0 | 0 | |
| 12 - 17 years | 33 | 33 | |
| Adults (18 - 64 years) | 245 | 245 | |
| From 65 - 84 years | 34 | 34 | |
| 85 years and over | 0 | 0 | |
| Age continuous | | | |
| Units: years | | | |
| arithmetic mean | 38.0 | | |
| standard deviation | ± 18.47 | - | |
| Gender categorical | | | |
| Units: Subjects | | | |
| Male | 167 | 167 | |
| Female | 145 | 145 | |
| Race | | | |
| Units: Subjects | | | |
| White | 217 | 217 | |
| Black or African American | 43 | 43 | |
| Asian | 44 | 44 | |
| Native Hawaiian or Other Pacific Islander | 3 | 3 | |
| Multiracial | 2 | 2 | |
| Not Reported | 3 | 3 | |
| Ethnicity | | | |
| Units: Subjects | | | |
| Hispanic or Latino | 43 | 43 | |
| Not Hispanic or Latino | 261 | 261 | |
| Not Reported | 8 | 8 | |

End points

End points reporting groups

| | |
|--|-------------|
| Reporting group title | Abrocitinib |
| Reporting group description: | |
| Participants received abrocitinib 100milligrams (mg) or 200 mg tablet orally once a day (QD) | |

Primary: Number of Participants With Treatment-Emergent Adverse Events (TEAEs) and Serious Adverse Events (SAEs)

| | |
|-----------------|--|
| End point title | Number of Participants With Treatment-Emergent Adverse Events (TEAEs) and Serious Adverse Events (SAEs) ^[1] |
|-----------------|--|

End point description:

An AE was any untoward medical occurrence in a participant or clinical study participant, temporally associated with use of study intervention, whether or not considered related to study intervention. An event was considered a TEAE if event started during the effective duration of treatment with investigational product. All events that started on or after first dosing day, but before or on the last dosing day plus lag time (28 days) were considered as TEAEs. SAE was an AE resulting in any of below outcomes or deemed significant for any other reason: death; initial or prolonged inpatient hospitalisation; life-threatening experience persistent or significant disability/incapacity; congenital anomaly; was a suspected transmission via a Pfizer product of an infectious agent, pathogenic or non-pathogenic or other situations where medical or scientific judgement should be exercised by investigator. AEs:SAEs and all non-SAEs. Safety set was analysed.

| | |
|----------------|---------|
| End point type | Primary |
|----------------|---------|

End point timeframe:

From the first dose of study treatment up to 4 weeks post end of study treatment (maximum exposure up to 155 weeks)

Notes:

[1] - No statistical analyses have been specified for this primary end point. It is expected there is at least one statistical analysis for each primary end point.

Justification: No statistical analysis have been defined.

| End point values | Abrocitinib | | | |
|-----------------------------|-----------------|--|--|--|
| Subject group type | Reporting group | | | |
| Number of subjects analysed | 312 | | | |
| Units: Participants | | | | |
| Participants with TEAEs | 244 | | | |
| Participants with SAEs | 17 | | | |

Statistical analyses

No statistical analyses for this end point

Primary: Number of Participants With Treatment-Emergent Serious Infections

| | |
|-----------------|--|
| End point title | Number of Participants With Treatment-Emergent Serious Infections ^[2] |
|-----------------|--|

End point description:

Serious infections, defined as any infection (viral, bacterial, and fungal) requiring hospitalisation or parenteral antimicrobials. Safety analysis set included all participants who received at least 1 dose of study intervention.

| | |
|----------------|---------|
| End point type | Primary |
|----------------|---------|

End point timeframe:

From the first dose of study treatment up to 4 weeks post end of study treatment (maximum exposure up to 155 weeks)

Notes:

[2] - No statistical analyses have been specified for this primary end point. It is expected there is at least one statistical analysis for each primary end point.

Justification: No statistical analysis have been defined.

| End point values | Abrocitinib | | | |
|-----------------------------|-----------------|--|--|--|
| Subject group type | Reporting group | | | |
| Number of subjects analysed | 312 | | | |
| Units: Participants | 4 | | | |

Statistical analyses

No statistical analyses for this end point

Primary: Number of Participants who Discontinued Study or Study Drug due to AEs and SAEs Respectively

| | |
|-----------------|---|
| End point title | Number of Participants who Discontinued Study or Study Drug due to AEs and SAEs Respectively ^[3] |
|-----------------|---|

End point description:

An AE is any untoward medical occurrence in a participant who received study treatment without regard to possibility of causal relationship. SAE is an AE resulting in any of the following outcomes or deemed significant for any other reason: death, initial or prolonged inpatient hospitalisation, life-threatening experience (immediate risk of dying), persistent or significant disability or incapacity, congenital anomaly. Number of participants who discontinued study and study drug due to AEs and SAEs were reported in this outcome measure. Safety analysis set included all participants who received at least 1 dose of study intervention.

| | |
|----------------|---------|
| End point type | Primary |
|----------------|---------|

End point timeframe:

From the first dose of study treatment up to 4 weeks post end of study treatment (maximum exposure up to 155 weeks)

Notes:

[3] - No statistical analyses have been specified for this primary end point. It is expected there is at least one statistical analysis for each primary end point.

Justification: No statistical analysis have been defined.

| End point values | Abrocitinib | | | |
|-------------------------------|-----------------|--|--|--|
| Subject group type | Reporting group | | | |
| Number of subjects analysed | 312 | | | |
| Units: Participants | | | | |
| Due to AE | 8 | | | |
| Due to AE and continue Study | 25 | | | |
| Due to SAE | 3 | | | |
| Due to SAE and continue Study | 7 | | | |

Statistical analyses

Adverse events

Adverse events information

Timeframe for reporting adverse events:

From the time of signing the informed consent up to 4 weeks post end of study treatment (maximum exposure up to 155 weeks)

Adverse event reporting additional description:

Same event may appear as both AE and SAE but are distinct events. An event may be categorised as serious in 1 participant and non-serious in another, or a participant may have experienced both SAE and non-SAE.

| | |
|-----------------|----------------|
| Assessment type | Non-systematic |
|-----------------|----------------|

Dictionary used

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

| | |
|--------------------|-------|
| Dictionary version | v27.0 |
|--------------------|-------|

Reporting groups

| | |
|-----------------------|-------------|
| Reporting group title | Abrocitinib |
|-----------------------|-------------|

Reporting group description:

Participants received abrocitinib 100 mg or 200 mg tablet orally once a day (QD)

| Serious adverse events | Abrocitinib | | |
|---|------------------|--|--|
| Total subjects affected by serious adverse events | | | |
| subjects affected / exposed | 17 / 312 (5.45%) | | |
| number of deaths (all causes) | 2 | | |
| number of deaths resulting from adverse events | 2 | | |
| Neoplasms benign, malignant and unspecified (incl cysts and polyps) | | | |
| Adenocarcinoma of colon | | | |
| subjects affected / exposed | 1 / 312 (0.32%) | | |
| occurrences causally related to treatment / all | 0 / 1 | | |
| deaths causally related to treatment / all | 0 / 0 | | |
| Immune system disorders | | | |
| Drug hypersensitivity | | | |
| subjects affected / exposed | 1 / 312 (0.32%) | | |
| occurrences causally related to treatment / all | 0 / 1 | | |
| deaths causally related to treatment / all | 0 / 0 | | |
| Respiratory, thoracic and mediastinal disorders | | | |
| Pulmonary oedema | | | |
| subjects affected / exposed | 1 / 312 (0.32%) | | |
| occurrences causally related to treatment / all | 0 / 1 | | |
| deaths causally related to treatment / all | 0 / 0 | | |

| | | | |
|---|-----------------|--|--|
| Pulmonary embolism | | | |
| subjects affected / exposed | 1 / 312 (0.32%) | | |
| occurrences causally related to treatment / all | 1 / 1 | | |
| deaths causally related to treatment / all | 0 / 0 | | |
| Dyspnoea | | | |
| subjects affected / exposed | 1 / 312 (0.32%) | | |
| occurrences causally related to treatment / all | 0 / 1 | | |
| deaths causally related to treatment / all | 0 / 0 | | |
| Acute respiratory distress syndrome | | | |
| subjects affected / exposed | 1 / 312 (0.32%) | | |
| occurrences causally related to treatment / all | 0 / 1 | | |
| deaths causally related to treatment / all | 0 / 0 | | |
| Psychiatric disorders | | | |
| Generalised anxiety disorder | | | |
| subjects affected / exposed | 1 / 312 (0.32%) | | |
| occurrences causally related to treatment / all | 0 / 1 | | |
| deaths causally related to treatment / all | 0 / 0 | | |
| Injury, poisoning and procedural complications | | | |
| Ligament rupture | | | |
| subjects affected / exposed | 1 / 312 (0.32%) | | |
| occurrences causally related to treatment / all | 0 / 1 | | |
| deaths causally related to treatment / all | 0 / 0 | | |
| Tendon injury | | | |
| subjects affected / exposed | 1 / 312 (0.32%) | | |
| occurrences causally related to treatment / all | 0 / 1 | | |
| deaths causally related to treatment / all | 0 / 0 | | |
| Tendon rupture | | | |
| subjects affected / exposed | 1 / 312 (0.32%) | | |
| occurrences causally related to treatment / all | 0 / 1 | | |
| deaths causally related to treatment / all | 0 / 0 | | |
| Cardiac disorders | | | |
| Hypertensive heart disease | | | |

| | | | |
|---|-----------------|--|--|
| subjects affected / exposed | 1 / 312 (0.32%) | | |
| occurrences causally related to treatment / all | 1 / 1 | | |
| deaths causally related to treatment / all | 1 / 1 | | |
| Nervous system disorders | | | |
| Syncope | | | |
| subjects affected / exposed | 1 / 312 (0.32%) | | |
| occurrences causally related to treatment / all | 0 / 1 | | |
| deaths causally related to treatment / all | 0 / 0 | | |
| Cerebral venous thrombosis | | | |
| subjects affected / exposed | 1 / 312 (0.32%) | | |
| occurrences causally related to treatment / all | 1 / 1 | | |
| deaths causally related to treatment / all | 0 / 0 | | |
| Blood and lymphatic system disorders | | | |
| Thrombocytopenia | | | |
| subjects affected / exposed | 1 / 312 (0.32%) | | |
| occurrences causally related to treatment / all | 1 / 1 | | |
| deaths causally related to treatment / all | 0 / 0 | | |
| Gastrointestinal disorders | | | |
| Gastroduodenal ulcer | | | |
| subjects affected / exposed | 1 / 312 (0.32%) | | |
| occurrences causally related to treatment / all | 0 / 1 | | |
| deaths causally related to treatment / all | 0 / 0 | | |
| Duodenal ulcer | | | |
| subjects affected / exposed | 1 / 312 (0.32%) | | |
| occurrences causally related to treatment / all | 1 / 1 | | |
| deaths causally related to treatment / all | 0 / 0 | | |
| Hepatobiliary disorders | | | |
| Hepatic function abnormal | | | |
| subjects affected / exposed | 1 / 312 (0.32%) | | |
| occurrences causally related to treatment / all | 0 / 1 | | |
| deaths causally related to treatment / all | 0 / 0 | | |
| Renal and urinary disorders | | | |
| Calculus urinary | | | |

| | | | |
|---|-----------------|--|--|
| subjects affected / exposed | 1 / 312 (0.32%) | | |
| occurrences causally related to treatment / all | 0 / 1 | | |
| deaths causally related to treatment / all | 0 / 0 | | |
| Infections and infestations | | | |
| Skin bacterial infection | | | |
| subjects affected / exposed | 1 / 312 (0.32%) | | |
| occurrences causally related to treatment / all | 0 / 1 | | |
| deaths causally related to treatment / all | 0 / 0 | | |
| Urosepsis | | | |
| subjects affected / exposed | 1 / 312 (0.32%) | | |
| occurrences causally related to treatment / all | 1 / 1 | | |
| deaths causally related to treatment / all | 0 / 0 | | |
| Renal abscess | | | |
| subjects affected / exposed | 1 / 312 (0.32%) | | |
| occurrences causally related to treatment / all | 1 / 1 | | |
| deaths causally related to treatment / all | 0 / 0 | | |
| Ophthalmic herpes zoster | | | |
| subjects affected / exposed | 1 / 312 (0.32%) | | |
| occurrences causally related to treatment / all | 1 / 1 | | |
| deaths causally related to treatment / all | 0 / 0 | | |
| COVID-19 pneumonia | | | |
| subjects affected / exposed | 1 / 312 (0.32%) | | |
| occurrences causally related to treatment / all | 0 / 1 | | |
| deaths causally related to treatment / all | 0 / 1 | | |
| COVID-19 | | | |
| subjects affected / exposed | 1 / 312 (0.32%) | | |
| occurrences causally related to treatment / all | 0 / 1 | | |
| deaths causally related to treatment / all | 0 / 0 | | |
| Metabolism and nutrition disorders | | | |
| Type 1 diabetes mellitus | | | |
| subjects affected / exposed | 1 / 312 (0.32%) | | |
| occurrences causally related to treatment / all | 0 / 1 | | |
| deaths causally related to treatment / all | 0 / 0 | | |

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

| Non-serious adverse events | Abrocitinib | | |
|---|--------------------|--|--|
| Total subjects affected by non-serious adverse events | | | |
| subjects affected / exposed | 162 / 312 (51.92%) | | |
| Nervous system disorders | | | |
| Headache | | | |
| subjects affected / exposed | 27 / 312 (8.65%) | | |
| occurrences (all) | 33 | | |
| Gastrointestinal disorders | | | |
| Nausea | | | |
| subjects affected / exposed | 48 / 312 (15.38%) | | |
| occurrences (all) | 54 | | |
| Skin and subcutaneous tissue disorders | | | |
| Dermatitis atopic | | | |
| subjects affected / exposed | 41 / 312 (13.14%) | | |
| occurrences (all) | 51 | | |
| Acne | | | |
| subjects affected / exposed | 28 / 312 (8.97%) | | |
| occurrences (all) | 29 | | |
| Infections and infestations | | | |
| Upper respiratory tract infection | | | |
| subjects affected / exposed | 21 / 312 (6.73%) | | |
| occurrences (all) | 26 | | |
| COVID-19 | | | |
| subjects affected / exposed | 51 / 312 (16.35%) | | |
| occurrences (all) | 54 | | |

More information

Substantial protocol amendments (globally)

Were there any global substantial amendments to the protocol? Yes

| Date | Amendment |
|------------------|---|
| 07 December 2020 | <p>The main reason for the protocol amendment was to update Appendix 2 The Table of Protocol Required Safety Laboratory tests to reflect minimally required clinical chemistry and hematology parameters to be tested. Other changes included clarifications to the following:</p> <ul style="list-style-type: none">• Hepatitis B testing• Exclusion criterion 15 to reflect that participants who are vaccinated with live components within 6 weeks prior to the first dose of Abrocitinib or who are expected to be vaccinated with these vaccines during treatment or during the 6 weeks following discontinuation of Abrocitinib are not eligible for study participation.• Lifestyle Considerations text regarding vaccination with live components has been updated to align with exclusion criterion 15 text• Permitted use of corticosteroid inhalers and intranasal sprays and ophthalmic corticosteroid to reflect that use of a stable dose of these permitted medications is not required.• Concomitant treatment monitoring to specify that monitoring of both medications and non-drug treatments will occur.• Rater Qualifications has been clarified.• Addition of reference regarding blood sample volumes in children added to the Study Assessments and Procedures section |

Notes:

Interruptions (globally)

Were there any global interruptions to the trial? No

Limitations and caveats

None reported