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 (gestional 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 to155 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 toAE	8			
Due to AE and continue Study	25			
Due to SAE	3			
Due to SAE and continue Study	7			

Statistical analyses

No statistical analyses for this end point

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	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: • 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