Clinical trial results:
Open-Label Extension Study to Evaluate the Safety of Long-Term Treatment with Avapritinib for Patients Previously Involved in an Avapritinib Study

Summary

EudraCT number	2020-005751-21
Trial protocol	FR
Global end of trial date	23 November 2023

Results information

Result version number	v1 (current)
This version publication date	01 December 2024
First version publication date	01 December 2024

Trial information

Trial identification

Sponsor protocol code	BLU-285-1408

Additional study identifiers

ISRCTN number	-
ClinicalTrials.gov id (NCT number)	NCT04825574
WHO universal trial number (UTN)	-

Notes:

Sponsors

Sponsor organisation name	Blueprint Medicines
Sponsor organisation address	45 Sidney Street, Cambridge, United States, 02139
Public contact	Blueprint Medicines Medical Information, Blueprint Medicines, 1 18882587768, medinfo@blueprintmedicines.com
Scientific contact	Blueprint Medicines Medical Information, Blueprint Medicines, 1 18882587768, medinfo@blueprintmedicines.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?	No

Notes:

Results analysis stage

Analysis stage	Final
Date of interim/final analysis	23 November 2023
Is this the analysis of the primary completion data?	No

Global end of trial reached?	Yes
Global end of trial date	23 November 2023
Was the trial ended prematurely?	No

Notes:

General information about the trial

Main objective of the trial:

This was an open-label extension study to provide long term safety data for GIST patients who were deriving clinical benefit from avapritinib upon the completion of avapritinib clinical trials.

Protection of trial subjects:

The study was conducted in accordance with ethical principles founded in the Declaration of Helsinki.

Background therapy: -

Evidence for comparator: -

Actual start date of recruitment	21 May 2021
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	France: 2
Worldwide total number of subjects	2
EEA total number of subjects	2

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)	0
Adults (18-64 years)	2
From 65 to 84 years	0
85 years and over	0

Subject disposition

Recruitment

Recruitment details: -

Pre-assignment

Screening details:

Two participants (one from BLU-285-1101 (NCT02508532) and one from BLU-285-1303 (NCT03465722)) were continuing to receive avapritinib and were enrolled in this extension study.

Period 1

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

Arms

Arm title	Avapritinib

Arm description:

Participants with gastrointestinal stromal tumor (GIST) who were deriving clinical benefit from avapritinib upon the completion of avapritinib clinical studies received avapritinib once daily (QD) at the specified dose of the prior avapritinib protocol.

Arm type	Experimental
Investigational medicinal product name	Avapritinib
Investigational medicinal product code	
Other name	BLU-285
Pharmaceutical forms	Tablet
Routes of administration	Oral use

Dosage and administration details:

Participants received avapritinib QD at the specified dose of the prior avapritinib protocol.

Number of subjects in period 1	Avapritinib
Started	2
Received at least 1 dose of study drug	2
Completed	0
Not completed	2
Disease progression	1
Commercial treatment became available	1

Baseline characteristics

Reporting groups	
Reporting group title	Avapritinib
Reporting group description:	
Participants with gastrointestinal stromal tumor (GIST) who were deriving clinical benefit from avapritinib upon the completion of avapritinib clinical studies received avapritinib once daily (QD) at the specified dose of the prior avapritinib protocol.	

Reporting group values	Avapritinib	Total	
Number of subjects	2	2	
Age Categorical Units: participants			
<=18 years	0	0	
Between 18 and 65 years	2	2	
>=65 years	0	0	
Sex: Female, Male Units: participants			
Not Reported for Participant Privacy	2	2	
Race (NIH/OMB) Units: Subjects			
Not Reported for Participant Privacy	2	2	

End points

End points reporting groups

Reporting group title	Avapritinib

Reporting group description:

Participants with gastrointestinal stromal tumor (GIST) who were deriving clinical benefit from avapritinib upon the completion of avapritinib clinical studies received avapritinib once daily (QD) at the specified dose of the prior avapritinib protocol.

Primary: Number of Participants with Adverse Events of Special Interest (AESIs)

End point title	Number of Participants with Adverse Events of Special Interest (AESIs)[1]

End point description:

AESIs for avapritinib are, regardless of grade or causality:
• cognitive effects which include the following terms: memory impairment, cognitive disorder, confusional state and encephalopathy.
• intracranial bleeding including haemorrhage intracranial, cerebral haemorrhage , and subdural haematoma.

The Safety Population was defined as all participants who received at least one dose of avapritinib.

End point type	Primary

End point timeframe:

From date of first avapritinib dose through 30 days after the last avapritinib dose (up to approximately 31 months)

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: Only summary statistics were planned for this outcome measure.

End point values	Avapritinib			
Subject group type	Reporting group			
Number of subjects analysed	2			
Units: participants	0			

Statistical analyses

No statistical analyses for this end point

Primary: Number of Participants with Serious Adverse Events (SAEs)

End point title	Number of Participants with Serious Adverse Events (SAEs)[2]

End point description:

An AE was defined as any untoward medical occurrence in a participant who received study drug without regard to possibility of causal relationship. SAEs were defined as death, a life-threatening AE, inpatient hospitalization or prolongation of existing hospitalization, persistent or significant disability or incapacity, a congenital anomaly or birth defect, or an important medical event that jeopardized participant and required medical intervention to prevent 1 of the outcomes listed in this definition. A summary of all serious AEs, regardless of causality is located in Reported AE section.

The Safety Population was defined as all participants who received at least one dose of avapritinib.

End point type	Primary

End point timeframe:

From date of first avapritinib dose through 30 days after the last avapritinib dose (up to approximately 31 months)

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: Only summary statistics were planned for this outcome measure.

End point values	Avapritinib			
Subject group type	Reporting group			
Number of subjects analysed	2			
Units: participants	1			

Statistical analyses

No statistical analyses for this end point

Adverse events

Adverse events information[1]

Timeframe for reporting adverse events:

From date of first avapritinib dose through 30 days after the last avapritinib dose (up to approximately 31 months)

Adverse event reporting additional description:

The Safety Population was defined as all participants who received at least one dose of avapritinib. As specified in the protocol, only SAEs and AESIs were collected. Non-serious adverse event data were not collected.

Assessment type	Systematic

Dictionary used

Dictionary name	MedDRA
Dictionary version	24.0

Reporting groups

Reporting group title	Avapritinib

Reporting group description:

Participants with GIST who were deriving clinical benefit from avapritinib upon the completion of avapritinib clinical studies received avapritinib QD at the specified dose of the prior avapritinib protocol.

Notes:

[1] - There are no non-serious adverse events recorded for these results. It is expected that there will be at least one non-serious adverse event reported.
Justification: As specified in the protocol, only SAEs and AESIs were collected. Non-serious adverse event data was not collected.

Serious adverse events	Avapritinib		
Total subjects affected by serious adverse events			
subjects affected / exposed	1 / 2 (50.00%)		
number of deaths (all causes)	0		
number of deaths resulting from adverse events			
Gastrointestinal disorders			
Gastrointestinal haemorrhage			
subjects affected / exposed	1 / 2 (50.00%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Vomiting			
subjects affected / exposed	1 / 2 (50.00%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Abdominal pain			
subjects affected / exposed	1 / 2 (50.00%)		
occurrences causally related to treatment / all	0 / 2		
deaths causally related to treatment / all	0 / 0		
Musculoskeletal and connective tissue disorders			

Arthralgia			
subjects affected / exposed	1 / 2 (50.00%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		

Frequency threshold for reporting non-serious adverse events: 0 %

Non-serious adverse events	Avapritinib		
Total subjects affected by non-serious adverse events			
subjects affected / exposed	0 / 2 (0.00%)		

More information

Substantial protocol amendments (globally)

Were there any global substantial amendments to the protocol? No

Interruptions (globally)

Were there any global interruptions to the trial? No

Limitations and caveats

None reported