



Clinical trial results:

A Phase 2, Open-Label, Monotherapy, Multicenter Study to Evaluate the Efficacy and Safety of INCB054828 in Subjects With Myeloid/Lymphoid Neoplasms With FGFR1 Rearrangement

Summary

EudraCT number	2016-002596-10
Trial protocol	GB DE AT ES BE IT
Global end of trial date	30 October 2024

Results information

Result version number	v1 (current)
This version publication date	20 November 2025
First version publication date	20 November 2025

Trial information

Trial identification

Sponsor protocol code	INCB 54828-203
-----------------------	----------------

Additional study identifiers

ISRCTN number	-
ClinicalTrials.gov id (NCT number)	NCT03011372
WHO universal trial number (UTN)	-

Notes:

Sponsors

Sponsor organisation name	Incyte Corporation
Sponsor organisation address	1801 Augustine Cutoff, Wilmington, United States, 19803
Public contact	Study Director, Incyte Corporation, 1 855-463-3463, medinfo@incyte.com
Scientific contact	Study Director, Incyte Corporation, 1 855-463-3463, medinfo@incyte.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	30 October 2024
Is this the analysis of the primary completion data?	No
Global end of trial reached?	Yes
Global end of trial date	30 October 2024
Was the trial ended prematurely?	No

Notes:

General information about the trial

Main objective of the trial:

The primary objective of this study was to evaluate the efficacy of pemigatinib in participants with myeloid/lymphoid neoplasms with fibroblast growth factor receptor (FGFR) 1 rearrangement.

Protection of trial subjects:

This study was performed in accordance with ethical principles that have their origin in the Declaration of Helsinki (Brazil 2013) and conducted in adherence to the study Protocol, applicable Good Clinical Practices, and applicable laws and country-specific regulations, including WMO (Medical Research Involving Human Participants Act) and Clinical Trials Regulation (European Union) No. 536/2014, in which the study was conducted.

Background therapy: -

Evidence for comparator: -

Actual start date of recruitment	25 April 2017
Long term follow-up planned	Yes
Long term follow-up rationale	Efficacy
Long term follow-up duration	24 Months
Independent data monitoring committee (IDMC) involvement?	No

Notes:

Population of trial subjects

Subjects enrolled per country

Country: Number of subjects enrolled	Belgium: 1
Country: Number of subjects enrolled	Canada: 1
Country: Number of subjects enrolled	France: 7
Country: Number of subjects enrolled	Germany: 7
Country: Number of subjects enrolled	Italy: 7
Country: Number of subjects enrolled	Japan: 2
Country: Number of subjects enrolled	Spain: 2
Country: Number of subjects enrolled	United Kingdom: 2
Country: Number of subjects enrolled	United States: 18
Worldwide total number of subjects	47
EEA total number of subjects	24

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)	29
From 65 to 84 years	18
85 years and over	0

Subject disposition

Recruitment

Recruitment details: -

Pre-assignment

Screening details:

This study was conducted at 21 study centers in Belgium, Canada, France, Germany, Italy, Japan, Spain, United Kingdom, and the United States.

Period 1

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

Arms

Arm title	Pemigatinib 13.5 mg
-----------	---------------------

Arm description:

Pemigatinib 13.5 milligrams (mg) was self-administered once daily (QD) as oral tablets on an intermittent dosing (ID) schedule or continuous dosing (CD) schedule. Participants started pemigatinib 13.5 mg on the ID schedule (i.e., 2 weeks on/1 week off) as per the initial Protocol and on the CD schedule in 21-day cycles following a Protocol amendment.

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

Dosage and administration details:

2 and 4.5 mg unit dose strength tablets

Number of subjects in period 1	Pemigatinib 13.5 mg
Started	47
Completed	0
Not completed	47
Consent withdrawn by subject	7
Moved to Commercial Supply of Investigational Drug	2
Received Chemotherapy for Stem Cell Transplant	1
Sponsor's Decision	2
Death	16
Sponsor Decision	17
Lost to follow-up	2

Baseline characteristics

Reporting groups

Reporting group title	Pemigatinib 13.5 mg
-----------------------	---------------------

Reporting group description:

Pemigatinib 13.5 milligrams (mg) was self-administered once daily (QD) as oral tablets on an intermittent dosing (ID) schedule or continuous dosing (CD) schedule. Participants started pemigatinib 13.5 mg on the ID schedule (i.e., 2 weeks on/1 week off) as per the initial Protocol and on the CD schedule in 21-day cycles following a Protocol amendment.

Reporting group values	Pemigatinib 13.5 mg	Total	
Number of subjects	47	47	
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	
Adolescents (12-17 years)	0	0	
Adults (18-64 years)	29	29	
From 65-84 years	18	18	
85 years and over	0	0	
Age Continuous			
Units: years			
arithmetic mean	58.3		
standard deviation	± 13.25	-	
Sex: Female, Male			
Units: participants			
Female	25	25	
Male	22	22	
Race/Ethnicity, Customized			
Units: Subjects			
White	30	30	
Black or African American	4	4	
Asian	4	4	
Missing	5	5	
Not Provided	2	2	
White/Caucasian and American-Indian Alaska Native	1	1	
African	1	1	
Race/Ethnicity, Customized			
Units: Subjects			
Hispanic or Latino	1	1	
Not Hispanic or Latino	33	33	
Not Reported	8	8	
Unknown	1	1	
Captured as "Other" in Database	4	4	

End points

End points reporting groups

Reporting group title	Pemigatinib 13.5 mg
Reporting group description: Pemigatinib 13.5 milligrams (mg) was self-administered once daily (QD) as oral tablets on an intermittent dosing (ID) schedule or continuous dosing (CD) schedule. Participants started pemigatinib 13.5 mg on the ID schedule (i.e., 2 weeks on/1 week off) as per the initial Protocol and on the CD schedule in 21-day cycles following a Protocol amendment.	

Primary: Percentage of participants who achieved complete response (CR) as determined by investigator assessment according to the response criteria for myeloid/lymphoid neoplasms with FGFR1 rearrangement

End point title	Percentage of participants who achieved complete response (CR) as determined by investigator assessment according to the response criteria for myeloid/lymphoid neoplasms with FGFR1 rearrangement ^[1]
-----------------	---

End point description:

CR was defined as the presence of all of the following improvements: (1) bone marrow: $\leq 5\%$ myeloblasts (including monocytic blast equivalent) and no lymphoblasts, with normal maturation of all cell lines, and return to age-adjusted normal cellularity; (2) osteomyelofibrosis absent or equal to "mild reticulatin fibrosis" (Grade 1 or less fibrosis); (3) peripheral blood: white blood cells (WBC) $\leq 10 \times 10^9$ cells/Liter (L); hemoglobin (Hgb) ≥ 11 grams per deciliter (g/dL); platelets $\geq 100 \times 10^9$ /L and $\leq 450 \times 10^9$ /L; neutrophils $\geq 1.0 \times 10^9$ /L; blasts = 0%; neutrophil precursors reduced to $\leq 2\%$; monocytes $\leq 1 \times 10^9$ /L; eosinophils $\leq 0.5 \times 10^9$ /L; (4) extramedullary disease: complete resolution of extramedullary disease present before therapy (e.g., lymphadenopathy), including palpable hepatosplenomegaly. Persistent low-level dysplasia was permitted given subjectivity of assignment of dysplasia. Response criteria by investigator assessment were the same for chronic phase (CP) and blast phase (BP).

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

End point timeframe:

up to 2513 days (120 21-day treatment cycles)

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: Statistical analysis was not conducted for this endpoint.

End point values	Pemigatinib 13.5 mg			
Subject group type	Reporting group			
Number of subjects analysed	45 ^[2]			
Units: percentage of participants				
number (confidence interval 95%)	68.9 (53.35 to 81.83)			

Notes:

[2] - Full Efficacy-Evaluable Population

Statistical analyses

No statistical analyses for this end point

Secondary: Percentage of participants who achieved a best overall response of complete response (CR) or partial response (PR) as determined by investigator and CRC assessment according to the response criteria for myeloid/lymphoid neoplasms

with FGFR1 rearrangement

End point title	Percentage of participants who achieved a best overall response of complete response (CR) or partial response (PR) as determined by investigator and CRC assessment according to the response criteria for myeloid/lymphoid neoplasms with FGFR1 rearrangement
-----------------	--

End point description:

CR=all of the following improvements: (1) bone marrow: $\leq 5\%$ myeloblasts (including monocytic blast equivalent) and no lymphoblasts, with normal maturation of all cell lines, and return to age-adjusted normal cellularity; (2) osteomyelofibrosis absent/equal to "mild reticulin fibrosis"; (3) WBC $\leq 10 \times 10^9$ cells/L; Hgb ≥ 11 g/dL; platelets $\geq 100 \times 10^9$ /L, $\leq 450 \times 10^9$ /L; neutrophils $\geq 1.0 \times 10^9$ /L; blasts=0%; neutrophil precursors reduced to $\leq 2\%$; monocytes $\leq 1 \times 10^9$ /L; eosinophils $\leq 0.5 \times 10^9$ /L; (4) extramedullary disease: complete resolution of extramedullary disease present pre-therapy, including palpable hepatosplenomegaly. Persistent low-level dysplasia was permitted. PR=all of the following improvements: (1) reduction of bone marrow blasts/blast equivalents by 50%, but remaining $>5\%$ of cellularity (except in cases with $\leq 5\%$ bone marrow blasts at baseline); (2) normalization of peripheral blood indices per CR Criterion 3; (3) extra medullary disease response of CMR/CR or PMR/PR.

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

End point timeframe:

up to 2513 days (120 21-day treatment cycles)

End point values	Pemigatinib 13.5 mg			
Subject group type	Reporting group			
Number of subjects analysed	45 ^[3]			
Units: percentage of participants				
number (confidence interval 95%)				
Investigator assessment	77.8 (62.91 to 88.80)			
CRC assessment	75.6 (60.46 to 87.12)			

Notes:

[3] - Full Efficacy-Evaluable Population

Statistical analyses

No statistical analyses for this end point

Secondary: Percentage of participants who achieved a complete cytogenetic response (CCyR) as assessed by local analysis and investigator evaluation and CRC assessment

End point title	Percentage of participants who achieved a complete cytogenetic response (CCyR) as assessed by local analysis and investigator evaluation and CRC assessment
-----------------	---

End point description:

CCyR was defined as 0% 8p11 translocated metaphases as seen on classic karyotyping with minimal of 20 metaphases, or fluorescence in situ hybridization (FISH). Loss of cytogenetic burden of disease (via FISH or classic karyotyping) was required to reach CCyR. Confidence intervals were calculated based on the exact method for binomial distribution.

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

End point timeframe:

up to 2513 days (120 21-day treatment cycles)

End point values	Pemigatinib 13.5 mg			
Subject group type	Reporting group			
Number of subjects analysed	45 ^[4]			
Units: percentage of participants				
number (confidence interval 95%)				
Investigator assessment	73.3 (58.06 to 85.40)			
CRC assessment	73.3 (58.06 to 85.40)			

Notes:

[4] - Full Efficacy-Evaluable Population

Statistical analyses

No statistical analyses for this end point

Secondary: Percentage of participants who achieved a partial cytogenetic response (PCyR) as assessed by local analysis and investigator evaluation and CRC assessment

End point title	Percentage of participants who achieved a partial cytogenetic response (PCyR) as assessed by local analysis and investigator evaluation and CRC assessment
-----------------	--

End point description:

PCyR was defined as the decrease from baseline of 50% or more 8p11 translocated metaphases as seen on classic karyotyping with minimal of 20 metaphases, or FISH.

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

End point timeframe:

up to 2513 days (120 21-day treatment cycles)

End point values	Pemigatinib 13.5 mg			
Subject group type	Reporting group			
Number of subjects analysed	45 ^[5]			
Units: percentage of participants				
number (confidence interval 95%)				
Investigator assessment	8.9 (2.48 to 21.22)			
CRC assessment	15.6 (6.49 to 29.46)			

Notes:

[5] - Full Efficacy-Evaluable Population

Statistical analyses

No statistical analyses for this end point

Secondary: Duration of complete response

End point title	Duration of complete response
-----------------	-------------------------------

End point description:

Duration of complete response was defined as the time from the first assessment of complete response to the earlier of the date of first worsening assessment after complete response or death due to any cause. Confidence intervals were calculated using the Brookmeyer and Crowley's method. 9999=The median and the upper limit of the confidence interval were not estimable because too few participants had worsening assessment after response or death.

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

End point timeframe:

up to 2513 days (120 21-day treatment cycles)

End point values	Pemigatinib 13.5 mg			
Subject group type	Reporting group			
Number of subjects analysed	31 ^[6]			
Units: months				
median (confidence interval 95%)				
Investigator assessment	53.29 (12.22 to 9999)			
CRC assessment	9999 (27.86 to 9999)			

Notes:

[6] - Full Efficacy-Evaluable Population. Only participants with a complete response were analyzed.

Statistical analyses

No statistical analyses for this end point

Secondary: Duration of response

End point title	Duration of response
-----------------	----------------------

End point description:

Duration of response was defined as the time from the first assessment of complete response or partial response to the earlier of the date of first worsening assessment after response or death due to any cause. Confidence intervals were calculated based on the exact method for binomial distribution. 9999=The median and the upper limit of the confidence interval were not estimable because too few participants had events of loss of response (disease progression) or death.

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

End point timeframe:

up to 2513 days (120 21-day treatment cycles)

End point values	Pemigatinib 13.5 mg			
Subject group type	Reporting group			
Number of subjects analysed	45 ^[7]			
Units: months				
median (confidence interval 95%)				
Investigator assessment, n=35	53.29 (15.51 to 9999)			

CRC assessment, n=34	9999 (27.86 to 9999)			
----------------------	----------------------	--	--	--

Notes:

[7] - Full Efficacy-Evaluable Population. Only participants with CR or PR were analyzed.

Statistical analyses

No statistical analyses for this end point

Secondary: Progression-free survival (PFS)

End point title	Progression-free survival (PFS)
End point description:	
PFS was defined as the time from the first date of taking study drug until the date of disease progression or until death due to any cause, whichever was earlier. Disease progression was defined as the combination of 2 major criteria, 1 major and 2 minor criteria, or 3 minor criteria from the following lists. Major criteria: (1) increase in blast count; (2) evidence of cytogenetic evolution (re-appearance of a previously present or appearance of a new cytogenetic abnormality, or increase in cytogenetic burden of disease); (3) new or worsening extramedullary disease (worsening splenomegaly or extramedullary disease outside of the spleen). Minor criteria: (1) transfusion dependence; (2) significant loss of maximal response on cytopenias $\geq 50\%$ decrement from maximum remission/response in granulocytes or platelets; (3) reduction in Hgb by $\geq 1.5\text{g/dL}$ from best response or from baseline as noted on complete blood count; (4) evidence of clonal evolution (molecular). 9999=not estimable.	
End point type	Secondary
End point timeframe:	
up to 2513 days (120 21-day treatment cycles)	

End point values	Pemigatinib 13.5 mg			
Subject group type	Reporting group			
Number of subjects analysed	45 ^[8]			
Units: months				
median (confidence interval 95%)				
Investigator assessment	73.89 (54.74 to 9999)			
CRC assessment	73.89 (29.17 to 9999)			

Notes:

[8] - Full Efficacy-Evaluable Population

Statistical analyses

No statistical analyses for this end point

Secondary: Overall survival

End point title	Overall survival
End point description:	
Overall survival was defined as as the time from the first day of taking study drug until death due to any cause. Confidence intervals were calculated using the Brookmeyer and Crowley's method. Participants without death observed at the time of the analysis were censored at the last date known to be alive. 9999=The median and the upper limit of the confidence interval were not estimable because too few participants died.	

End point type	Secondary
End point timeframe: up to 2513 days (120 21-day treatment cycles)	

End point values	Pemigatinib 13.5 mg			
Subject group type	Reporting group			
Number of subjects analysed	45 ^[9]			
Units: months				
median (confidence interval 95%)	9999 (54.74 to 9999)			

Notes:

[9] - Full Efficacy-Evaluable Population

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:

An adverse event (AE) was defined as any untoward medical occurrence associated with the use of a drug in humans, whether or not considered drug related, that occurs after a participant provides informed consent. A TEAE was defined as any AE either reported for the first time or the worsening of a pre-existing event after the first dose of study drug.

End point type	Secondary
End point timeframe: up to 2543 days	

End point values	Pemigatinib 13.5 mg			
Subject group type	Reporting group			
Number of subjects analysed	47 ^[10]			
Units: participants	47			

Notes:

[10] - Safety Population: all enrolled participants who received at least 1 dose of study drug

Statistical analyses

No statistical analyses for this end point

Secondary: Number of participants with any ≥Grade 3 TEAE

End point title	Number of participants with any ≥Grade 3 TEAE
-----------------	---

End point description:

An AE was defined as any untoward medical occurrence associated with the use of a drug in humans,

whether or not considered drug related, that occurs after a participant provides informed consent. A TEAE was defined as any AE either reported for the first time or the worsening of a pre-existing event after the first dose of study drug. The severity of AEs was assessed using Common Terminology Criteria for Adverse Events (CTCAE) version 4.03 Grades 1 through 4. Grade 1: mild; asymptomatic or mild symptoms; clinical or diagnostic observations only; intervention not indicated. Grade 2: moderate; minimal, local, or noninvasive intervention indicated; limiting age-appropriate activities of daily living. Grade 3: Severe or medically significant but not immediately life-threatening; hospitalization or prolongation of hospitalization indicated; disabling; limiting self-care activities of daily living. Grade 4: life-threatening consequences; urgent intervention indicated.

End point type	Secondary
End point timeframe:	
up to 2543 days	

End point values	Pemigatinib 13.5 mg			
Subject group type	Reporting group			
Number of subjects analysed	47 ^[11]			
Units: participants	39			

Notes:

[11] - Safety Population

Statistical analyses

No statistical analyses for this end point

Other pre-specified: Percentage of participants who achieved CR as determined by central review committee (CRC) assessment

End point title	Percentage of participants who achieved CR as determined by central review committee (CRC) assessment
-----------------	---

End point description:

In addition, responses were assessed by CRC based on Myeloid/Lymphoid Neoplasm International Working Group (MLN IWG) response criteria for myeloid/lymphoid neoplasms with eosinophilia and tyrosine kinase gene fusions. Confidence intervals were calculated based on the exact method for binomial distribution.

End point type	Other pre-specified
End point timeframe:	
up to 2513 days (120 21-day treatment cycles)	

End point values	Pemigatinib 13.5 mg			
Subject group type	Reporting group			
Number of subjects analysed	45 ^[12]			
Units: percentage of participants				
number (confidence interval 95%)	68.9 (53.35 to 81.83)			

Notes:

[12] - Full Efficacy-Evaluable Population

Statistical analyses

No statistical analyses for this end point

Other pre-specified: Percentage of participants who achieved a best overall response of CR or PR as determined by CRC assessment

End point title	Percentage of participants who achieved a best overall response of CR or PR as determined by CRC assessment
End point description: In addition, responses were assessed by CRC based on MLN IWG response criteria for myeloid/lymphoid neoplasms with eosinophilia and tyrosine kinase gene fusions. Confidence intervals were calculated based on the exact method for binomial distribution.	
End point type	Other pre-specified
End point timeframe: up to 2513 days (120 21-day treatment cycles)	

End point values	Pemigatinib 13.5 mg			
Subject group type	Reporting group			
Number of subjects analysed	45 ^[13]			
Units: percentage of participants				
number (confidence interval 95%)	75.6 (60.46 to 87.12)			

Notes:

[13] - Full Efficacy-Evaluable Population

Statistical analyses

No statistical analyses for this end point

Other pre-specified: Percentage of participants who achieved a CCyR as assessed by CRC assessment

End point title	Percentage of participants who achieved a CCyR as assessed by CRC assessment
End point description: In addition, responses were assessed by CRC based on MLN IWG response criteria for myeloid/lymphoid neoplasms with eosinophilia and tyrosine kinase gene fusions. Confidence intervals were calculated based on the exact method for binomial distribution.	
End point type	Other pre-specified
End point timeframe: up to 2513 days (120 21-day treatment cycles)	

End point values	Pemigatinib 13.5 mg			
Subject group type	Reporting group			
Number of subjects analysed	45 ^[14]			
Units: percentage of participants				
number (confidence interval 95%)	73.3 (58.06 to 85.40)			

Notes:

[14] - Full Efficacy-Evaluable Population

Statistical analyses

No statistical analyses for this end point

Other pre-specified: Percentage of participants who achieved a PCyR as assessed by CRC assessment

End point title	Percentage of participants who achieved a PCyR as assessed by CRC assessment
-----------------	--

End point description:

In addition, responses were assessed by CRC based on MLN IWG response criteria for myeloid/lymphoid neoplasms with eosinophilia and tyrosine kinase gene fusions. Confidence intervals were calculated based on the exact method for binomial distribution.

End point type	Other pre-specified
----------------	---------------------

End point timeframe:

up to 2513 days (120 21-day treatment cycles)

End point values	Pemigatinib 13.5 mg			
Subject group type	Reporting group			
Number of subjects analysed	45 ^[15]			
Units: percentage of participants				
number (confidence interval 95%)	15.6 (6.49 to 29.46)			

Notes:

[15] - Full Efficacy-Evaluable Population

Statistical analyses

No statistical analyses for this end point

Adverse events

Adverse events information

Timeframe for reporting adverse events:

up to 2730 days

Adverse event reporting additional description:

Adverse events have been reported for the Safety Population, comprised of all enrolled participants who received at least 1 dose of study drug.

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

Dictionary used

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

Dictionary version	22
--------------------	----

Reporting groups

Reporting group title	Pemigatinib 13.5 mg
-----------------------	---------------------

Reporting group description:

Pemigatinib 13.5 milligrams (mg) was self-administered once daily (QD) as oral tablets on an intermittent dosing (ID) schedule or continuous dosing (CD) schedule. Participants started pemigatinib 13.5 mg on the ID schedule (i.e., 2 weeks on/1 week off) as per the initial Protocol and on the CD schedule in 21-day cycles following a Protocol amendment.

Serious adverse events	Pemigatinib 13.5 mg		
Total subjects affected by serious adverse events			
subjects affected / exposed	27 / 47 (57.45%)		
number of deaths (all causes)	16		
number of deaths resulting from adverse events	6		
Neoplasms benign, malignant and unspecified (incl cysts and polyps)			
Malignant neoplasm progression			
subjects affected / exposed	1 / 47 (2.13%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 1		
Vascular disorders			
Aortic stenosis			
subjects affected / exposed	1 / 47 (2.13%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
General disorders and administration site conditions			
Chest pain			
subjects affected / exposed	1 / 47 (2.13%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		

General physical health deterioration			
subjects affected / exposed	1 / 47 (2.13%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Pain			
subjects affected / exposed	1 / 47 (2.13%)		
occurrences causally related to treatment / all	1 / 1		
deaths causally related to treatment / all	0 / 0		
Multiple organ dysfunction syndrome			
subjects affected / exposed	1 / 47 (2.13%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 1		
Pyrexia			
subjects affected / exposed	1 / 47 (2.13%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Reproductive system and breast disorders			
Oedema genital			
subjects affected / exposed	1 / 47 (2.13%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Respiratory, thoracic and mediastinal disorders			
Pulmonary embolism			
subjects affected / exposed	1 / 47 (2.13%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Psychiatric disorders			
Depression			
subjects affected / exposed	1 / 47 (2.13%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Confusional state			

subjects affected / exposed	1 / 47 (2.13%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Investigations			
Hepatic enzyme increased			
subjects affected / exposed	1 / 47 (2.13%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Injury, poisoning and procedural complications			
Hip fracture			
subjects affected / exposed	1 / 47 (2.13%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Humerus fracture			
subjects affected / exposed	1 / 47 (2.13%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Femur fracture			
subjects affected / exposed	1 / 47 (2.13%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Fall			
subjects affected / exposed	1 / 47 (2.13%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Intentional overdose			
subjects affected / exposed	1 / 47 (2.13%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Subdural haematoma			
subjects affected / exposed	1 / 47 (2.13%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		

Skin abrasion			
subjects affected / exposed	1 / 47 (2.13%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Cardiac disorders			
Angina pectoris			
subjects affected / exposed	2 / 47 (4.26%)		
occurrences causally related to treatment / all	0 / 2		
deaths causally related to treatment / all	0 / 0		
Atrial flutter			
subjects affected / exposed	1 / 47 (2.13%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Atrioventricular block complete			
subjects affected / exposed	2 / 47 (4.26%)		
occurrences causally related to treatment / all	0 / 2		
deaths causally related to treatment / all	0 / 0		
Heart failure with preserved ejection fraction			
subjects affected / exposed	1 / 47 (2.13%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Myocardial infarction			
subjects affected / exposed	2 / 47 (4.26%)		
occurrences causally related to treatment / all	0 / 2		
deaths causally related to treatment / all	0 / 1		
Nervous system disorders			
Syncope			
subjects affected / exposed	2 / 47 (4.26%)		
occurrences causally related to treatment / all	1 / 3		
deaths causally related to treatment / all	0 / 0		
Seizure			
subjects affected / exposed	1 / 47 (2.13%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		

Gastrointestinal disorders			
Diarrhoea			
subjects affected / exposed	1 / 47 (2.13%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Upper gastrointestinal haemorrhage			
subjects affected / exposed	1 / 47 (2.13%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Hepatobiliary disorders			
Cholecystitis			
subjects affected / exposed	1 / 47 (2.13%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Hypertransaminasaemia			
subjects affected / exposed	1 / 47 (2.13%)		
occurrences causally related to treatment / all	1 / 1		
deaths causally related to treatment / all	0 / 0		
Skin and subcutaneous tissue disorders			
Alopecia			
subjects affected / exposed	1 / 47 (2.13%)		
occurrences causally related to treatment / all	1 / 1		
deaths causally related to treatment / all	0 / 0		
Renal and urinary disorders			
Acute kidney injury			
subjects affected / exposed	4 / 47 (8.51%)		
occurrences causally related to treatment / all	1 / 4		
deaths causally related to treatment / all	0 / 1		
Bladder tamponade			
subjects affected / exposed	1 / 47 (2.13%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Bladder obstruction			

subjects affected / exposed	1 / 47 (2.13%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Nephrolithiasis			
subjects affected / exposed	1 / 47 (2.13%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Postrenal failure			
subjects affected / exposed	1 / 47 (2.13%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Urinary retention			
subjects affected / exposed	1 / 47 (2.13%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Musculoskeletal and connective tissue disorders			
Spinal pain			
subjects affected / exposed	1 / 47 (2.13%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Spinal flattening			
subjects affected / exposed	1 / 47 (2.13%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Infections and infestations			
Erysipelas			
subjects affected / exposed	1 / 47 (2.13%)		
occurrences causally related to treatment / all	2 / 2		
deaths causally related to treatment / all	0 / 0		
Endocarditis			
subjects affected / exposed	1 / 47 (2.13%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 1		

Corneal abscess				
subjects affected / exposed	1 / 47 (2.13%)			
occurrences causally related to treatment / all	1 / 1			
deaths causally related to treatment / all	0 / 0			
Pneumonia pseudomonal				
subjects affected / exposed	1 / 47 (2.13%)			
occurrences causally related to treatment / all	1 / 1			
deaths causally related to treatment / all	0 / 0			
Pneumonia aspiration				
subjects affected / exposed	1 / 47 (2.13%)			
occurrences causally related to treatment / all	0 / 1			
deaths causally related to treatment / all	0 / 0			
Pneumonia				
subjects affected / exposed	2 / 47 (4.26%)			
occurrences causally related to treatment / all	0 / 2			
deaths causally related to treatment / all	0 / 0			
Infection				
subjects affected / exposed	1 / 47 (2.13%)			
occurrences causally related to treatment / all	0 / 1			
deaths causally related to treatment / all	0 / 1			
Urinary tract infection				
subjects affected / exposed	2 / 47 (4.26%)			
occurrences causally related to treatment / all	0 / 2			
deaths causally related to treatment / all	0 / 0			
Pyelonephritis				
subjects affected / exposed	1 / 47 (2.13%)			
occurrences causally related to treatment / all	0 / 1			
deaths causally related to treatment / all	0 / 0			
Stenotrophomonas infection				
subjects affected / exposed	1 / 47 (2.13%)			
occurrences causally related to treatment / all	0 / 1			
deaths causally related to treatment / all	0 / 0			
Sepsis				

subjects affected / exposed	1 / 47 (2.13%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Pseudomonas infection			
subjects affected / exposed	1 / 47 (2.13%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Metabolism and nutrition disorders			
Calciphylaxis			
subjects affected / exposed	1 / 47 (2.13%)		
occurrences causally related to treatment / all	1 / 1		
deaths causally related to treatment / all	0 / 0		
Hypercalcaemia			
subjects affected / exposed	1 / 47 (2.13%)		
occurrences causally related to treatment / all	0 / 2		
deaths causally related to treatment / all	0 / 0		
Hyperphosphataemia			
subjects affected / exposed	1 / 47 (2.13%)		
occurrences causally related to treatment / all	1 / 1		
deaths causally related to treatment / all	0 / 0		

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

Non-serious adverse events	Pemigatinib 13.5 mg		
Total subjects affected by non-serious adverse events			
subjects affected / exposed	46 / 47 (97.87%)		
Vascular disorders			
Hypertension			
subjects affected / exposed	5 / 47 (10.64%)		
occurrences (all)	8		
Hypotension			
subjects affected / exposed	4 / 47 (8.51%)		
occurrences (all)	6		
General disorders and administration site conditions			

Chills			
subjects affected / exposed	4 / 47 (8.51%)		
occurrences (all)	5		
Asthenia			
subjects affected / exposed	10 / 47 (21.28%)		
occurrences (all)	12		
Fatigue			
subjects affected / exposed	11 / 47 (23.40%)		
occurrences (all)	15		
Oedema peripheral			
subjects affected / exposed	11 / 47 (23.40%)		
occurrences (all)	15		
Non-cardiac chest pain			
subjects affected / exposed	3 / 47 (6.38%)		
occurrences (all)	3		
Pyrexia			
subjects affected / exposed	10 / 47 (21.28%)		
occurrences (all)	19		
Respiratory, thoracic and mediastinal disorders			
Cough			
subjects affected / exposed	9 / 47 (19.15%)		
occurrences (all)	11		
Dyspnoea			
subjects affected / exposed	10 / 47 (21.28%)		
occurrences (all)	15		
Epistaxis			
subjects affected / exposed	16 / 47 (34.04%)		
occurrences (all)	23		
Oropharyngeal pain			
subjects affected / exposed	9 / 47 (19.15%)		
occurrences (all)	12		
Nasal congestion			
subjects affected / exposed	5 / 47 (10.64%)		
occurrences (all)	7		
Upper-airway cough syndrome			

subjects affected / exposed occurrences (all)	3 / 47 (6.38%) 3		
Psychiatric disorders			
Anxiety			
subjects affected / exposed	5 / 47 (10.64%)		
occurrences (all)	7		
Confusional state			
subjects affected / exposed	3 / 47 (6.38%)		
occurrences (all)	3		
Depression			
subjects affected / exposed	3 / 47 (6.38%)		
occurrences (all)	3		
Insomnia			
subjects affected / exposed	5 / 47 (10.64%)		
occurrences (all)	8		
Investigations			
Alanine aminotransferase increased			
subjects affected / exposed	6 / 47 (12.77%)		
occurrences (all)	11		
Blood creatinine increased			
subjects affected / exposed	8 / 47 (17.02%)		
occurrences (all)	11		
Blood alkaline phosphatase increased			
subjects affected / exposed	11 / 47 (23.40%)		
occurrences (all)	14		
Aspartate aminotransferase increased			
subjects affected / exposed	6 / 47 (12.77%)		
occurrences (all)	10		
Weight decreased			
subjects affected / exposed	8 / 47 (17.02%)		
occurrences (all)	9		
Injury, poisoning and procedural complications			
Contusion			
subjects affected / exposed	6 / 47 (12.77%)		
occurrences (all)	8		
Fall			

subjects affected / exposed occurrences (all)	4 / 47 (8.51%) 10		
Cardiac disorders Palpitations subjects affected / exposed occurrences (all)	3 / 47 (6.38%) 3		
Nervous system disorders Ageusia subjects affected / exposed occurrences (all) Dizziness subjects affected / exposed occurrences (all) Dysgeusia subjects affected / exposed occurrences (all) Headache subjects affected / exposed occurrences (all) Hypogeusia subjects affected / exposed occurrences (all) Paraesthesia subjects affected / exposed occurrences (all) Neuropathy peripheral subjects affected / exposed occurrences (all) Memory impairment subjects affected / exposed occurrences (all) Tremor subjects affected / exposed occurrences (all)	6 / 47 (12.77%) 7 9 / 47 (19.15%) 16 7 / 47 (14.89%) 7 12 / 47 (25.53%) 15 3 / 47 (6.38%) 3 3 / 47 (6.38%) 3 3 / 47 (6.38%) 3 6 / 47 (12.77%) 6 3 / 47 (6.38%) 3		
Blood and lymphatic system disorders			

Anaemia			
subjects affected / exposed	16 / 47 (34.04%)		
occurrences (all)	27		
Leukopenia			
subjects affected / exposed	5 / 47 (10.64%)		
occurrences (all)	6		
Neutropenia			
subjects affected / exposed	4 / 47 (8.51%)		
occurrences (all)	9		
Thrombocytopenia			
subjects affected / exposed	6 / 47 (12.77%)		
occurrences (all)	8		
Eye disorders			
Conjunctival hyperaemia			
subjects affected / exposed	3 / 47 (6.38%)		
occurrences (all)	3		
Corneal erosion			
subjects affected / exposed	3 / 47 (6.38%)		
occurrences (all)	3		
Detachment of retinal pigment epithelium			
subjects affected / exposed	3 / 47 (6.38%)		
occurrences (all)	3		
Dry eye			
subjects affected / exposed	20 / 47 (42.55%)		
occurrences (all)	22		
Blepharitis			
subjects affected / exposed	4 / 47 (8.51%)		
occurrences (all)	4		
Cataract			
subjects affected / exposed	5 / 47 (10.64%)		
occurrences (all)	5		
Entropion			
subjects affected / exposed	3 / 47 (6.38%)		
occurrences (all)	3		
Epiretinal membrane			

subjects affected / exposed	3 / 47 (6.38%)		
occurrences (all)	4		
Eye discharge			
subjects affected / exposed	3 / 47 (6.38%)		
occurrences (all)	3		
Eye pain			
subjects affected / exposed	3 / 47 (6.38%)		
occurrences (all)	3		
Keratitis			
subjects affected / exposed	5 / 47 (10.64%)		
occurrences (all)	6		
Lacrimation increased			
subjects affected / exposed	8 / 47 (17.02%)		
occurrences (all)	9		
Photophobia			
subjects affected / exposed	3 / 47 (6.38%)		
occurrences (all)	3		
Macular oedema			
subjects affected / exposed	3 / 47 (6.38%)		
occurrences (all)	5		
Punctate keratitis			
subjects affected / exposed	3 / 47 (6.38%)		
occurrences (all)	3		
Visual acuity reduced			
subjects affected / exposed	3 / 47 (6.38%)		
occurrences (all)	3		
Vision blurred			
subjects affected / exposed	10 / 47 (21.28%)		
occurrences (all)	10		
Trichiasis			
subjects affected / exposed	7 / 47 (14.89%)		
occurrences (all)	8		
Subretinal fluid			
subjects affected / exposed	3 / 47 (6.38%)		
occurrences (all)	3		
Gastrointestinal disorders			

Abdominal distension			
subjects affected / exposed	3 / 47 (6.38%)		
occurrences (all)	6		
Aphthous ulcer			
subjects affected / exposed	3 / 47 (6.38%)		
occurrences (all)	3		
Abdominal pain			
subjects affected / exposed	16 / 47 (34.04%)		
occurrences (all)	25		
Abdominal pain upper			
subjects affected / exposed	7 / 47 (14.89%)		
occurrences (all)	9		
Dyspepsia			
subjects affected / exposed	10 / 47 (21.28%)		
occurrences (all)	11		
Constipation			
subjects affected / exposed	20 / 47 (42.55%)		
occurrences (all)	29		
Diarrhoea			
subjects affected / exposed	29 / 47 (61.70%)		
occurrences (all)	51		
Dry mouth			
subjects affected / exposed	19 / 47 (40.43%)		
occurrences (all)	20		
Dysphagia			
subjects affected / exposed	3 / 47 (6.38%)		
occurrences (all)	3		
Gastrooesophageal reflux disease			
subjects affected / exposed	3 / 47 (6.38%)		
occurrences (all)	5		
Haemorrhoids			
subjects affected / exposed	5 / 47 (10.64%)		
occurrences (all)	5		
Oral pain			
subjects affected / exposed	3 / 47 (6.38%)		
occurrences (all)	4		

Nausea			
subjects affected / exposed	17 / 47 (36.17%)		
occurrences (all)	19		
Mouth ulceration			
subjects affected / exposed	5 / 47 (10.64%)		
occurrences (all)	6		
Vomiting			
subjects affected / exposed	7 / 47 (14.89%)		
occurrences (all)	19		
Stomatitis			
subjects affected / exposed	21 / 47 (44.68%)		
occurrences (all)	51		
Hepatobiliary disorders			
Hypertransaminasaemia			
subjects affected / exposed	4 / 47 (8.51%)		
occurrences (all)	13		
Skin and subcutaneous tissue disorders			
Alopecia			
subjects affected / exposed	26 / 47 (55.32%)		
occurrences (all)	29		
Dermatitis			
subjects affected / exposed	3 / 47 (6.38%)		
occurrences (all)	3		
Dry skin			
subjects affected / exposed	12 / 47 (25.53%)		
occurrences (all)	13		
Erythema			
subjects affected / exposed	6 / 47 (12.77%)		
occurrences (all)	7		
Hyperhidrosis			
subjects affected / exposed	4 / 47 (8.51%)		
occurrences (all)	6		
Hyperkeratosis			
subjects affected / exposed	3 / 47 (6.38%)		
occurrences (all)	4		
Ingrowing nail			

subjects affected / exposed	3 / 47 (6.38%)		
occurrences (all)	5		
Palmar-plantar erythrodysaesthesia syndrome			
subjects affected / exposed	7 / 47 (14.89%)		
occurrences (all)	16		
Onychomadesis			
subjects affected / exposed	8 / 47 (17.02%)		
occurrences (all)	8		
Onycholysis			
subjects affected / exposed	8 / 47 (17.02%)		
occurrences (all)	9		
Onychoclasia			
subjects affected / exposed	4 / 47 (8.51%)		
occurrences (all)	4		
Onychalgia			
subjects affected / exposed	5 / 47 (10.64%)		
occurrences (all)	6		
Nail bed tenderness			
subjects affected / exposed	3 / 47 (6.38%)		
occurrences (all)	4		
Nail discolouration			
subjects affected / exposed	4 / 47 (8.51%)		
occurrences (all)	4		
Nail disorder			
subjects affected / exposed	4 / 47 (8.51%)		
occurrences (all)	5		
Nail dystrophy			
subjects affected / exposed	8 / 47 (17.02%)		
occurrences (all)	8		
Skin exfoliation			
subjects affected / exposed	5 / 47 (10.64%)		
occurrences (all)	5		
Rash			
subjects affected / exposed	12 / 47 (25.53%)		
occurrences (all)	19		

Pruritus			
subjects affected / exposed	6 / 47 (12.77%)		
occurrences (all)	9		
Skin ulcer			
subjects affected / exposed	5 / 47 (10.64%)		
occurrences (all)	9		
Renal and urinary disorders			
Acute kidney injury			
subjects affected / exposed	3 / 47 (6.38%)		
occurrences (all)	5		
Dysuria			
subjects affected / exposed	4 / 47 (8.51%)		
occurrences (all)	5		
Pollakiuria			
subjects affected / exposed	3 / 47 (6.38%)		
occurrences (all)	3		
Musculoskeletal and connective tissue disorders			
Back pain			
subjects affected / exposed	7 / 47 (14.89%)		
occurrences (all)	14		
Arthralgia			
subjects affected / exposed	10 / 47 (21.28%)		
occurrences (all)	20		
Flank pain			
subjects affected / exposed	3 / 47 (6.38%)		
occurrences (all)	3		
Pain in extremity			
subjects affected / exposed	13 / 47 (27.66%)		
occurrences (all)	16		
Neck pain			
subjects affected / exposed	5 / 47 (10.64%)		
occurrences (all)	6		
Muscle spasms			
subjects affected / exposed	7 / 47 (14.89%)		
occurrences (all)	7		
Muscular weakness			

subjects affected / exposed	4 / 47 (8.51%)		
occurrences (all)	4		
Musculoskeletal pain			
subjects affected / exposed	5 / 47 (10.64%)		
occurrences (all)	5		
Myalgia			
subjects affected / exposed	6 / 47 (12.77%)		
occurrences (all)	9		
Infections and infestations			
Conjunctivitis			
subjects affected / exposed	5 / 47 (10.64%)		
occurrences (all)	7		
Cystitis			
subjects affected / exposed	4 / 47 (8.51%)		
occurrences (all)	12		
Candida infection			
subjects affected / exposed	3 / 47 (6.38%)		
occurrences (all)	3		
COVID-19			
subjects affected / exposed	3 / 47 (6.38%)		
occurrences (all)	3		
Bronchitis			
subjects affected / exposed	3 / 47 (6.38%)		
occurrences (all)	3		
Erysipelas			
subjects affected / exposed	3 / 47 (6.38%)		
occurrences (all)	5		
Fungal infection			
subjects affected / exposed	3 / 47 (6.38%)		
occurrences (all)	3		
Pharyngitis			
subjects affected / exposed	6 / 47 (12.77%)		
occurrences (all)	6		
Paronychia			
subjects affected / exposed	3 / 47 (6.38%)		
occurrences (all)	3		

Oral herpes			
subjects affected / exposed	3 / 47 (6.38%)		
occurrences (all)	3		
Oral candidiasis			
subjects affected / exposed	3 / 47 (6.38%)		
occurrences (all)	3		
Nasopharyngitis			
subjects affected / exposed	3 / 47 (6.38%)		
occurrences (all)	3		
Pneumonia			
subjects affected / exposed	3 / 47 (6.38%)		
occurrences (all)	4		
Urinary tract infection			
subjects affected / exposed	7 / 47 (14.89%)		
occurrences (all)	11		
Upper respiratory tract infection			
subjects affected / exposed	3 / 47 (6.38%)		
occurrences (all)	3		
Sinusitis			
subjects affected / exposed	4 / 47 (8.51%)		
occurrences (all)	7		
Metabolism and nutrition disorders			
Decreased appetite			
subjects affected / exposed	16 / 47 (34.04%)		
occurrences (all)	19		
Dehydration			
subjects affected / exposed	3 / 47 (6.38%)		
occurrences (all)	3		
Hypercalcaemia			
subjects affected / exposed	7 / 47 (14.89%)		
occurrences (all)	8		
Hyperglycaemia			
subjects affected / exposed	3 / 47 (6.38%)		
occurrences (all)	3		
Hyperphosphataemia			

subjects affected / exposed	35 / 47 (74.47%)		
occurrences (all)	59		
Hyperuricaemia			
subjects affected / exposed	3 / 47 (6.38%)		
occurrences (all)	3		
Hypoalbuminaemia			
subjects affected / exposed	3 / 47 (6.38%)		
occurrences (all)	6		
Hypocalcaemia			
subjects affected / exposed	4 / 47 (8.51%)		
occurrences (all)	5		
Hypokalaemia			
subjects affected / exposed	7 / 47 (14.89%)		
occurrences (all)	14		
Hypomagnesaemia			
subjects affected / exposed	3 / 47 (6.38%)		
occurrences (all)	5		
Hyponatraemia			
subjects affected / exposed	3 / 47 (6.38%)		
occurrences (all)	8		
Hypophosphataemia			
subjects affected / exposed	5 / 47 (10.64%)		
occurrences (all)	7		
Vitamin D deficiency			
subjects affected / exposed	5 / 47 (10.64%)		
occurrences (all)	6		

More information

Substantial protocol amendments (globally)

Were there any global substantial amendments to the protocol? Yes

Date	Amendment
09 August 2016	The primary purpose of this Protocol Amendment was to provide additional clinical data from the ongoing INCB 53828-101 study, refine the inclusion criteria to better define the population, and to amend the pharmacokinetic (PK) and electrocardiogram (ECG) sampling timepoint. requirements
12 December 2016	The primary purpose of this amendment was to update language based on Regulatory Agencies' comments. Updates included but were not limited to clarification of inclusion and exclusion criteria and guidance for dose reductions.
17 May 2018	The purpose of this amendment was to add language to allow for continuous administration of INCB054828. Updated clinical data were added to support continuous administration. Other modifications were made based on new preclinical and/or clinical data.
22 May 2019	The main purpose of this amendment was to modify the primary and secondary study efficacy endpoints and to revise the proposed response criteria. Other modifications were made to include treatment-naïve participants and to update the Protocol with program-level standard language.
02 July 2020	The main purpose of this amendment was to include updated language for comprehensive eye examination, per Regulatory Agency feedback. Other modifications were made to include a long-term treatment visit schedule option for participants with stable response, to update the Protocol with program-level standard language and post-transplant follow-up, and to provide additional language clarifications.
13 July 2023	The main purpose of this amendment was to modify the long-term treatment visit schedule to start at Cycle 18 and to update the assessments in the post-transplant follow-up period. This amendment also incorporated changes from country-specific adaptations.

Notes:

Interruptions (globally)

Were there any global interruptions to the trial? No

Limitations and caveats

None reported