



## Clinical trial results:

### A Phase 2, Open-Label, Single-Arm, Multicenter Study to Evaluate the Efficacy and Safety of Pemigatinib in Participants With Previously Treated Glioblastoma or Other Primary Central Nervous System Tumors Harboring Activating FGFR1 3 Alterations (FIGHT-209)

#### Summary

EudraCT number	2021-004740-24
Trial protocol	DE ES DK FR IT NL
Global end of trial date	17 December 2024

#### Results information

Result version number	v1 (current)
This version publication date	25 December 2025
First version publication date	25 December 2025

#### Trial information

##### Trial identification

Sponsor protocol code	INCB 54828-209
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##### Additional study identifiers

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

Notes:

#### Sponsors

Sponsor organisation name	Incyte Corporation
Sponsor organisation address	1801 Augustine Cutoff, 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	17 December 2024
Is this the analysis of the primary completion data?	No
Global end of trial reached?	Yes
Global end of trial date	17 December 2024
Was the trial ended prematurely?	Yes

Notes:

## General information about the trial

Main objective of the trial:

This study was conducted to determine the efficacy of pemigatinib in participants with recurrent glioblastoma with an activating fibroblast growth factor receptor (FGFR)1-3 mutation or fusion/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	20 May 2022
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	Denmark: 3
Country: Number of subjects enrolled	France: 11
Country: Number of subjects enrolled	Germany: 2
Country: Number of subjects enrolled	Italy: 17
Country: Number of subjects enrolled	Japan: 2
Country: Number of subjects enrolled	Netherlands: 3
Country: Number of subjects enrolled	Spain: 15
Country: Number of subjects enrolled	United Kingdom: 9
Country: Number of subjects enrolled	United States: 21
Worldwide total number of subjects	83
EEA total number of subjects	51

Notes:

### Subjects enrolled per age group

In utero	0
Preterm newborn - gestational age < 37	0

wk	
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)	64
From 65 to 84 years	19
85 years and over	0

## Subject disposition

### Recruitment

Recruitment details: -

### Pre-assignment

Screening details:

This study was conducted at 31 study centers in Denmark, France, Germany, Italy, Japan, Netherlands, Spain, the 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

Are arms mutually exclusive?	Yes
<b>Arm title</b>	Recurrent glioblastoma

Arm description:

Participants with recurrent glioblastoma with defined activating fibroblast growth factor receptor (FGFR) gene alterations received pemigatinib 13.5 milligrams (mg) once daily (QD) on a 2-week on-therapy and 1-week off-therapy schedule as long as they were receiving benefit and had not met any criteria for treatment discontinuation. Defined activating gene alterations included FGFR 1-3 fusions or rearrangements with intact FGFR kinase domain, or a defined set of FGFR 1-3 activating mutations or in-frame deletions.

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:

Unit dose strength(s)/dosage level(s): 13.5 mg, 9 mg, and 4.5 mg

<b>Arm title</b>	Recurrent non-glioblastoma CNS tumors
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Arm description:

Participants with recurrent non-glioblastoma central nervous system (CNS) tumors with defined activating FGFR gene alterations received pemigatinib 13.5 milligrams (mg) once daily (QD) on a 2-week on-therapy and 1-week off-therapy schedule as long as they were receiving benefit and had not met any criteria for treatment discontinuation. Defined activating gene alterations included FGFR 1-3 fusions or rearrangements with intact FGFR kinase domain, or a defined set of FGFR 1-3 activating mutations or in-frame deletions.

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:

Unit dose strength(s)/dosage level(s): 13.5 mg, 9 mg, and 4.5 mg

Number of subjects in period 1	Recurrent glioblastoma	Recurrent non-glioblastoma CNS tumors
Started	74	9
Completed	0	0
Not completed	74	9
Consent withdrawn by subject	-	1
Study terminated by Sponsor	24	6
Death	50	2

## Baseline characteristics

### Reporting groups

Reporting group title	Recurrent glioblastoma
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Reporting group description:

Participants with recurrent glioblastoma with defined activating fibroblast growth factor receptor (FGFR) gene alterations received pemigatinib 13.5 milligrams (mg) once daily (QD) on a 2-week on-therapy and 1-week off-therapy schedule as long as they were receiving benefit and had not met any criteria for treatment discontinuation. Defined activating gene alterations included FGFR 1-3 fusions or rearrangements with intact FGFR kinase domain, or a defined set of FGFR 1-3 activating mutations or in-frame deletions.

Reporting group title	Recurrent non-glioblastoma CNS tumors
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Reporting group description:

Participants with recurrent non-glioblastoma central nervous system (CNS) tumors with defined activating FGFR gene alterations received pemigatinib 13.5 milligrams (mg) once daily (QD) on a 2-week on-therapy and 1-week off-therapy schedule as long as they were receiving benefit and had not met any criteria for treatment discontinuation. Defined activating gene alterations included FGFR 1-3 fusions or rearrangements with intact FGFR kinase domain, or a defined set of FGFR 1-3 activating mutations or in-frame deletions.

Reporting group values	Recurrent glioblastoma	Recurrent non-glioblastoma CNS tumors	Total
Number of subjects	74	9	83
Age categorical			
Units: Subjects			
In utero	0	0	0
Preterm newborn infants (gestational age < 37 wks)	0	0	0
Newborns (0-27 days)	0	0	0
Infants and toddlers (28 days-23 months)	0	0	0
Children (2-11 years)	0	0	0
Adolescents (12-17 years)	0	0	0
Adults (18-64 years)	55	9	64
From 65-84 years	19	0	19
85 years and over	0	0	0
Age Continuous			
Units: years			
arithmetic mean	57.0	39.2	
standard deviation	± 11.45	± 12.75	-
Sex: Female, Male			
Units: participants			
Female	30	3	33
Male	44	6	50
Race/Ethnicity, Customized			
Units: Subjects			
White/Caucasian	58	6	64
Black/African-American	2	0	2
Asian	2	0	2
Not Reported	7	1	8
Unknown	5	1	6
Captured as "Other" in Database	0	1	1

Ethnicity (NIH/OMB)			
Units: Subjects			
Hispanic or Latino	1	0	1
Not Hispanic or Latino	60	6	66
Unknown or Not Reported	13	3	16

## End points

### End points reporting groups

Reporting group title	Recurrent glioblastoma
Reporting group description: Participants with recurrent glioblastoma with defined activating fibroblast growth factor receptor (FGFR) gene alterations received pemigatinib 13.5 milligrams (mg) once daily (QD) on a 2-week on-therapy and 1-week off-therapy schedule as long as they were receiving benefit and had not met any criteria for treatment discontinuation. Defined activating gene alterations included FGFR 1-3 fusions or rearrangements with intact FGFR kinase domain, or a defined set of FGFR 1-3 activating mutations or in-frame deletions.	
Reporting group title	Recurrent non-glioblastoma CNS tumors
Reporting group description: Participants with recurrent non-glioblastoma central nervous system (CNS) tumors with defined activating FGFR gene alterations received pemigatinib 13.5 milligrams (mg) once daily (QD) on a 2-week on-therapy and 1-week off-therapy schedule as long as they were receiving benefit and had not met any criteria for treatment discontinuation. Defined activating gene alterations included FGFR 1-3 fusions or rearrangements with intact FGFR kinase domain, or a defined set of FGFR 1-3 activating mutations or in-frame deletions.	

### Primary: Objective response rate (ORR) in participants with recurrent glioblastoma based on Independent Central Review

End point title	Objective response rate (ORR) in participants with recurrent glioblastoma based on Independent Central Review <sup>[1]</sup>
End point description: ORR=percentage of participants with a best overall response of complete response (CR) or partial response (PR) based on Response Assessment in Neuro-Oncology (RANO), determined by an independent centralized radiological review committee. CR requires: disappearance of all enhancing measurable/nonmeasurable disease for ≥4 weeks; no new lesions; stable/improved nonenhancing lesions; off corticosteroids/on physiologic replacement doses only and stable/improved clinically. PR requires: ≥50% decrease, compared with baseline (BL), in the sum of products of perpendicular diameters of measurable enhancing lesions sustained for ≥4 weeks; no progression of nonmeasurable disease; no new lesions; stable or improved nonenhancing lesions on same/lower dose of corticosteroids compared with BL scan; on a corticosteroid dose not greater than the dose at time of BL scan and stable/improved clinically. The 95% confidence interval (CI) was calculated based on the exact method for binomial distribution.	
End point type	Primary
End point timeframe: up to 651 days	

#### 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	Recurrent glioblastoma	Recurrent non-glioblastoma CNS tumors		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	74 <sup>[2]</sup>	0 <sup>[3]</sup>		
Units: percentage of participants				
number (confidence interval 95%)				
Confirmed tumor responses	5.4 (1.49 to 13.27)	( to )		
Unconfirmed tumor responses	8.1 (3.03 to 16.82)	( to )		

Notes:

[2] - Full Analysis Set: all enrolled participants who received  $\geq 1$  dose of pemigatinib

[3] - Full Analysis Set: all enrolled participants who received  $\geq 1$  dose of pemigatinib

## Statistical analyses

No statistical analyses for this end point

### Secondary: ORR in participants with recurrent non-glioblastoma central nervous system tumors based on Independent Central Review

End point title	ORR in participants with recurrent non-glioblastoma central nervous system tumors based on Independent Central Review
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End point description:

ORR was defined as the percentage of participants who achieved a best overall response of CR or PR based on RANO as determined by an independent centralized radiological review committee. CR requires all of the following: disappearance of all enhancing measurable/nonmeasurable disease sustained for  $\geq 4$  weeks; no new lesions; stable/improved nonenhancing lesions; off corticosteroids/on physiologic replacement doses only and stable/improved clinically. PR requires all of the following:  $\geq 50\%$  decrease, compared with baseline, in the sum of products of perpendicular diameters of all measurable enhancing lesions sustained for  $\geq 4$  weeks; no progression of nonmeasurable disease; no new lesions; stable or improved nonenhancing lesions on same/lower dose of corticosteroids compared with baseline scan; on a corticosteroid dose not greater than the dose at time of baseline scan and stable/improved clinically.

End point type	Secondary
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End point timeframe:

up to 784 days

End point values	Recurrent glioblastoma	Recurrent non-glioblastoma CNS tumors		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	0 <sup>[4]</sup>	9 <sup>[5]</sup>		
Units: percentage of participants				
number (confidence interval 95%)				
Confirmed tumor responses	( to )	22.2 (2.81 to 60.01)		
Unconfirmed tumor responses	( to )	22.2 (2.81 to 60.01)		

Notes:

[4] - Full Analysis Set. The 95% CI was calculated based on the exact method for binomial distribution.

[5] - Full Analysis Set. The 95% CI was calculated based on the exact method for binomial distribution.

## Statistical analyses

No statistical analyses for this end point

### Secondary: Duration of confirmed response based on Independent Central Review

End point title	Duration of confirmed response based on Independent Central Review
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End point description:

Duration of response=time from first assessment of confirmed CR/PR until progressive disease (PD) (according to RANO per an independent centralized radiological review committee) or death (whichever

occurred first). Progression was any of:  $\geq 25\%$  increase in sum of products of perpendicular diameters of enhancing lesions (compared with BL if no decrease) on stable/increasing doses of corticosteroids; a significant increase in T2/FLAIR nonenhancing lesions on stable/increasing doses of corticosteroids compared with BL scan or best response after initiation of therapy, not due to comorbid events; the appearance of any new lesions; clear progression of nonmeasurable lesions; or definite clinical deterioration not attributable to other causes apart from the tumor or to decrease in corticosteroid dose. The 95% CIs were calculated using the Brookmeyer and Crowley method. -9999, 9999=Values were not estimable because no responders had disease progression or died.

End point type	Secondary
End point timeframe: up to 784 days	

End point values	Recurrent glioblastoma	Recurrent non-glioblastoma CNS tumors		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	4 <sup>[6]</sup>	2 <sup>[7]</sup>		
Units: months				
median (confidence interval 95%)	9999 (-9999 to 9999)	9999 (-9999 to 9999)		

Notes:

[6] - Full Analysis Set. Only those participants with a confirmed CR or PR were analyzed.

[7] - Full Analysis Set. Only those participants with a confirmed CR or PR were analyzed.

## Statistical analyses

No statistical analyses for this end point

## Secondary: Duration of unconfirmed response based on Independent Central Review

End point title	Duration of unconfirmed response based on Independent Central Review
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End point description:

PFS was the time from the first dose until progressive disease (according to RANO per an independent centralized radiological review committee) or death (whichever occurred first). Progression was any of:  $\geq 25\%$  increase in sum of products of perpendicular diameters of enhancing lesions (compared with BL if no decrease) on stable/increasing doses of corticosteroids; a significant increase in T2/FLAIR nonenhancing lesions on stable/increasing doses of corticosteroids compared with BL scan or best response after initiation of therapy, not due to comorbid events; the appearance of any new lesions; clear progression of nonmeasurable lesions; or definite clinical deterioration not attributable to other causes apart from the tumor or to decrease in corticosteroid dose. The 95% CIs were calculated using the Brookmeyer and Crowley method. -9999, 9999=Values were not estimable because too few participants had disease progression or died.

End point type	Secondary
End point timeframe: up to 784 days	

End point values	Recurrent glioblastoma	Recurrent non-glioblastoma CNS tumors		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	6 <sup>[8]</sup>	2 <sup>[9]</sup>		
Units: months				
median (confidence interval 95%)	9999 (1.74 to 9999)	9999 (-9999 to 9999)		

Notes:

[8] - Full Analysis Set. Only those participants with an unconfirmed CR or PR were analyzed.

[9] - Full Analysis Set. Only those participants with an unconfirmed CR or PR were analyzed.

## Statistical analyses

No statistical analyses for this end point

## Secondary: ORR as determined by investigator assessment

End point title	ORR as determined by investigator assessment
End point description:	
ORR was defined as the percentage of participants who achieved an unconfirmed best overall response of CR or PR based on RANO as determined by investigator assessment. CR requires all of the following: disappearance of all enhancing measurable/nonmeasurable disease sustained for $\geq 4$ weeks; no new lesions; stable/improved nonenhancing lesions; off corticosteroids/on physiologic replacement doses only and stable/improved clinically. PR requires all of the following: $\geq 50\%$ decrease, compared with baseline, in the sum of products of perpendicular diameters of all measurable enhancing lesions sustained for $\geq 4$ weeks; no progression of nonmeasurable disease; no new lesions; stable or improved nonenhancing lesions on same/lower dose of corticosteroids compared with baseline scan; on a corticosteroid dose not greater than the dose at time of baseline scan and stable/improved clinically. The 95% CIs were calculated based on the exact method for binomial distribution.	
End point type	Secondary
End point timeframe:	
up to 784 days	

End point values	Recurrent glioblastoma	Recurrent non-glioblastoma CNS tumors		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	74 <sup>[10]</sup>	9 <sup>[11]</sup>		
Units: percentage of participants				
number (confidence interval 95%)	8.1 (3.03 to 16.82)	22.2 (2.81 to 60.01)		

Notes:

[10] - Full Analysis Set

[11] - Full Analysis Set

## 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)
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**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 it was considered drug related. An AE could therefore have been any unfavorable or unintended sign (including an abnormal laboratory finding), symptom, or disease (new or exacerbated) temporally associated with the use of study drug. A TEAE was defined as an AE reported for the first time or the worsening of a pre-existing event after the first dose of pemigatinib and within 30 days of the last dose of pemigatinib.

End point type	Secondary
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End point timeframe:

up to 814 days

End point values	Recurrent glioblastoma	Recurrent non-glioblastoma CNS tumors		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	74 <sup>[12]</sup>	9 <sup>[13]</sup>		
Units: participants	73	9		

Notes:

[12] - Safety Population: all enrolled participants who received at least 1 dose of pemigatinib

[13] - Safety Population: all enrolled participants who received at least 1 dose of pemigatinib

## Statistical analyses

No statistical analyses for this end point

## Secondary: Number of participants with TEAEs leading to discontinuation of pemigatinib, pemigatinib dose interruption, and pemigatinib dose reduction

End point title	Number of participants with TEAEs leading to discontinuation of pemigatinib, pemigatinib dose interruption, and pemigatinib dose reduction
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**End point description:**

An AE was defined as any untoward medical occurrence associated with the use of a drug in humans, whether or not it was considered drug related. An AE could therefore have been any unfavorable or unintended sign (including an abnormal laboratory finding), symptom, or disease (new or exacerbated) temporally associated with the use of study drug. A TEAE was defined as an AE reported for the first time or the worsening of a pre-existing event after the first dose of pemigatinib and within 30 days of the last dose of pemigatinib.

End point type	Secondary
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End point timeframe:

up to 814 days

End point values	Recurrent glioblastoma	Recurrent non-glioblastoma CNS tumors		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	74 <sup>[14]</sup>	9 <sup>[15]</sup>		
Units: participants				
TEAEs leading to discontinuation of pemigatinib	2	0		
TEAEs leading to pemigatinib dose interruption	22	4		

TEAEs leading to pemigatinib dose reduction	5	2		
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Notes:

[14] - Safety Population

[15] - Safety Population

## 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 it was considered drug related. A TEAE was defined as an AE reported for the first time or the worsening of a pre-existing event after the first dose of pemigatinib and within 30 days of the last dose of pemigatinib. The severity of AEs was assessed using Common Terminology Criteria for Adverse Events (CTCAE) version 5.0 Grades 1 through 5. Grade 1: mild; asymptomatic or mild symptoms; clinical or diagnostic observations only; treatment not indicated. Grade 2: moderate; minimal, local, or noninvasive treatment 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 treatment indicated. Grade 5: fatal.	
End point type	Secondary
End point timeframe:	
up to 814 days	

End point values	Recurrent glioblastoma	Recurrent non-glioblastoma CNS tumors		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	74 <sup>[16]</sup>	9 <sup>[17]</sup>		
Units: participants	27	3		

Notes:

[16] - Safety Population

[17] - Safety Population

## Statistical analyses

No statistical analyses for this end point

### Secondary: Disease control rate (DCR) based on Independent Central Review

End point title	Disease control rate (DCR) based on Independent Central Review
End point description:	
DCR was defined as the percentage of participants who achieved a best overall response of CR, PR, or stable disease (SD) based on RANO as determined by an independent centralized radiological review committee. In the case of SD, measurements must have met the SD criteria after the date of the first dose at a minimum interval of 42 days. SD occurred if the participant didn't qualify for CR, PR, or PD and required the following: stable nonenhancing (T2/FLAIR) lesions on same or lower dose of corticosteroids compared with baseline scan and clinically stable status. The 95% CIs were calculated based on the exact method for binomial distribution.	
End point type	Secondary

End point timeframe:  
up to 784 days

End point values	Recurrent glioblastoma	Recurrent non-glioblastoma CNS tumors		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	74 <sup>[18]</sup>	9 <sup>[19]</sup>		
Units: percentage of participants				
number (confidence interval 95%)				
Confirmed tumor responses	36.5 (25.60 to 48.49)	66.7 (29.93 to 92.51)		
Unconfirmed tumor responses	36.5 (25.60 to 48.49)	66.7 (29.93 to 92.51)		

Notes:

[18] - Full Analysis Set

[19] - Full Analysis Set

### Statistical analyses

No statistical analyses for this end point

### Secondary: Progression-free survival (PFS) based on Independent Central Review

End point title	Progression-free survival (PFS) based on Independent Central Review
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End point description:

PFS was the time from the first dose until progressive disease (according to RANO and assessed by an independent centralized radiological review committee) or death (whichever occurred first). Progression was any of:  $\geq 25\%$  increase in sum of products of perpendicular diameters of enhancing lesions (compared with BL if no decrease) on stable/increasing doses of corticosteroids; a significant increase in T2/FLAIR nonenhancing lesions on stable/increasing doses of corticosteroids compared with BL scan or best response after initiation of therapy, not due to comorbid events; the appearance of any new lesions; clear progression of nonmeasurable lesions; or definite clinical deterioration not attributable to other causes apart from the tumor or to decrease in corticosteroid dose. The 95% CIs were calculated using the Brookmeyer and Crowley method. 9999=The median and the upper bound of the confidence interval were not estimable because too few participants had disease progression or died.

End point type	Secondary
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End point timeframe:

up to 784 days

End point values	Recurrent glioblastoma	Recurrent non-glioblastoma CNS tumors		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	74 <sup>[20]</sup>	9 <sup>[21]</sup>		
Units: months				
median (confidence interval 95%)	2.07 (2.04 to 2.37)	9999 (1.74 to 9999)		

Notes:

[20] - Full Analysis Set

**Statistical analyses**

No statistical analyses for this end point

**Secondary: Overall survival**

End point title	Overall survival
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End point description:

Overall survival was defined as the time from the first dose of study drug to death due to any cause. The 95% CIs were calculated using the Brookmeyer and Crowley method. 9999=The upper bound of the confidence interval was not estimable because too few participants died.

End point type	Secondary
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End point timeframe:

up to 784 days

End point values	Recurrent glioblastoma	Recurrent non-glioblastoma CNS tumors		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	74 <sup>[22]</sup>	9 <sup>[23]</sup>		
Units: months				
median (confidence interval 95%)	11.37 (9.23 to 14.85)	24.08 (18.79 to 9999)		

Notes:

[22] - Full Analysis Set

[23] - Full Analysis Set

**Statistical analyses**

No statistical analyses for this end point

## Adverse events

### Adverse events information

Timeframe for reporting adverse events:

up to 814 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 pemigatinib.

Assessment type	Systematic
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### Dictionary used

Dictionary name	MedDRA
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Dictionary version	27.1
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### Reporting groups

Reporting group title	Recurrent glioblastoma
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Reporting group description:

Participants with recurrent glioblastoma with defined activating fibroblast growth factor receptor (FGFR) gene alterations received pemigatinib 13.5 milligrams (mg) once daily (QD) on a 2-week on-therapy and 1-week off-therapy schedule as long as they were receiving benefit and had not met any criteria for treatment discontinuation. Defined activating gene alterations included FGFR 1-3 fusions or rearrangements with intact FGFR kinase domain, or a defined set of FGFR 1-3 activating mutations or in-frame deletions.

Reporting group title	Total
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Reporting group description:

Total

Reporting group title	Recurrent non-glioblastoma CNS tumors
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Reporting group description:

Participants with recurrent non-glioblastoma central nervous system (CNS) tumors with defined activating FGFR gene alterations received pemigatinib 13.5 milligrams (mg) once daily (QD) on a 2-week on-therapy and 1-week off-therapy schedule as long as they were receiving benefit and had not met any criteria for treatment discontinuation. Defined activating gene alterations included FGFR 1-3 fusions or rearrangements with intact FGFR kinase domain, or a defined set of FGFR 1-3 activating mutations or in-frame deletions.

Serious adverse events	Recurrent glioblastoma	Total	Recurrent non-glioblastoma CNS tumors
Total subjects affected by serious adverse events			
subjects affected / exposed	17 / 74 (22.97%)	17 / 83 (20.48%)	0 / 9 (0.00%)
number of deaths (all causes)	50	52	2
number of deaths resulting from adverse events	1	1	0
Injury, poisoning and procedural complications			
Fall			
subjects affected / exposed	1 / 74 (1.35%)	1 / 83 (1.20%)	0 / 9 (0.00%)
occurrences causally related to treatment / all	0 / 1	0 / 1	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Lumbar vertebral fracture			

subjects affected / exposed	1 / 74 (1.35%)	1 / 83 (1.20%)	0 / 9 (0.00%)
occurrences causally related to treatment / all	0 / 1	0 / 1	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Stroke-like migraine attacks after radiation therapy			
subjects affected / exposed	1 / 74 (1.35%)	1 / 83 (1.20%)	0 / 9 (0.00%)
occurrences causally related to treatment / all	0 / 1	0 / 1	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Wound dehiscence			
subjects affected / exposed	1 / 74 (1.35%)	1 / 83 (1.20%)	0 / 9 (0.00%)
occurrences causally related to treatment / all	0 / 2	0 / 2	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Nervous system disorders			
Cognitive disorder			
subjects affected / exposed	1 / 74 (1.35%)	1 / 83 (1.20%)	0 / 9 (0.00%)
occurrences causally related to treatment / all	0 / 1	0 / 1	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Aphasia			
subjects affected / exposed	1 / 74 (1.35%)	1 / 83 (1.20%)	0 / 9 (0.00%)
occurrences causally related to treatment / all	0 / 1	0 / 1	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Epilepsy			
subjects affected / exposed	1 / 74 (1.35%)	1 / 83 (1.20%)	0 / 9 (0.00%)
occurrences causally related to treatment / all	0 / 1	0 / 1	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Hemiparesis			
subjects affected / exposed	2 / 74 (2.70%)	2 / 83 (2.41%)	0 / 9 (0.00%)
occurrences causally related to treatment / all	0 / 2	0 / 2	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Haemorrhage intracranial			
subjects affected / exposed	1 / 74 (1.35%)	1 / 83 (1.20%)	0 / 9 (0.00%)
occurrences causally related to treatment / all	0 / 1	0 / 1	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Partial seizures with secondary generalisation			

subjects affected / exposed	1 / 74 (1.35%)	1 / 83 (1.20%)	0 / 9 (0.00%)
occurrences causally related to treatment / all	0 / 1	0 / 1	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Seizure			
subjects affected / exposed	2 / 74 (2.70%)	2 / 83 (2.41%)	0 / 9 (0.00%)
occurrences causally related to treatment / all	0 / 2	0 / 2	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
General disorders and administration site conditions			
Asthenia			
subjects affected / exposed	1 / 74 (1.35%)	1 / 83 (1.20%)	0 / 9 (0.00%)
occurrences causally related to treatment / all	0 / 1	0 / 1	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Gastrointestinal disorders			
Faecaloma			
subjects affected / exposed	1 / 74 (1.35%)	1 / 83 (1.20%)	0 / 9 (0.00%)
occurrences causally related to treatment / all	0 / 1	0 / 1	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Intestinal perforation			
subjects affected / exposed	1 / 74 (1.35%)	1 / 83 (1.20%)	0 / 9 (0.00%)
occurrences causally related to treatment / all	0 / 1	0 / 1	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Respiratory, thoracic and mediastinal disorders			
Pulmonary embolism			
subjects affected / exposed	1 / 74 (1.35%)	1 / 83 (1.20%)	0 / 9 (0.00%)
occurrences causally related to treatment / all	0 / 1	0 / 1	0 / 0
deaths causally related to treatment / all	0 / 1	0 / 1	0 / 0
Renal and urinary disorders			
Acute kidney injury			
subjects affected / exposed	1 / 74 (1.35%)	1 / 83 (1.20%)	0 / 9 (0.00%)
occurrences causally related to treatment / all	0 / 1	0 / 1	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Psychiatric disorders			
Confusional state			

subjects affected / exposed	2 / 74 (2.70%)	2 / 83 (2.41%)	0 / 9 (0.00%)
occurrences causally related to treatment / all	0 / 2	0 / 2	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
<b>Infections and infestations</b>			
Herpes zoster			
subjects affected / exposed	1 / 74 (1.35%)	1 / 83 (1.20%)	0 / 9 (0.00%)
occurrences causally related to treatment / all	1 / 1	1 / 1	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Pneumonia			
subjects affected / exposed	2 / 74 (2.70%)	2 / 83 (2.41%)	0 / 9 (0.00%)
occurrences causally related to treatment / all	0 / 2	0 / 2	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Sepsis			
subjects affected / exposed	1 / 74 (1.35%)	1 / 83 (1.20%)	0 / 9 (0.00%)
occurrences causally related to treatment / all	0 / 1	0 / 1	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Urinary tract infection			
subjects affected / exposed	2 / 74 (2.70%)	2 / 83 (2.41%)	0 / 9 (0.00%)
occurrences causally related to treatment / all	1 / 2	1 / 2	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0

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

<b>Non-serious adverse events</b>	Recurrent glioblastoma	Total	Recurrent non-glioblastoma CNS tumors
Total subjects affected by non-serious adverse events			
subjects affected / exposed	71 / 74 (95.95%)	80 / 83 (96.39%)	9 / 9 (100.00%)
<b>Vascular disorders</b>			
Embolism			
subjects affected / exposed	0 / 74 (0.00%)	1 / 83 (1.20%)	1 / 9 (11.11%)
occurrences (all)	0	1	1
Hypertension			
subjects affected / exposed	5 / 74 (6.76%)	5 / 83 (6.02%)	0 / 9 (0.00%)
occurrences (all)	6	6	0
<b>General disorders and administration site conditions</b>			

Asthenia			
subjects affected / exposed	10 / 74 (13.51%)	12 / 83 (14.46%)	2 / 9 (22.22%)
occurrences (all)	17	20	3
Fatigue			
subjects affected / exposed	21 / 74 (28.38%)	24 / 83 (28.92%)	3 / 9 (33.33%)
occurrences (all)	23	26	3
Influenza like illness			
subjects affected / exposed	0 / 74 (0.00%)	1 / 83 (1.20%)	1 / 9 (11.11%)
occurrences (all)	0	1	1
Pyrexia			
subjects affected / exposed	5 / 74 (6.76%)	6 / 83 (7.23%)	1 / 9 (11.11%)
occurrences (all)	6	7	1
Temperature regulation disorder			
subjects affected / exposed	0 / 74 (0.00%)	1 / 83 (1.20%)	1 / 9 (11.11%)
occurrences (all)	0	1	1
Respiratory, thoracic and mediastinal disorders			
Cough			
subjects affected / exposed	5 / 74 (6.76%)	6 / 83 (7.23%)	1 / 9 (11.11%)
occurrences (all)	5	6	1
Epistaxis			
subjects affected / exposed	2 / 74 (2.70%)	4 / 83 (4.82%)	2 / 9 (22.22%)
occurrences (all)	2	4	2
Nasal dryness			
subjects affected / exposed	0 / 74 (0.00%)	2 / 83 (2.41%)	2 / 9 (22.22%)
occurrences (all)	0	2	2
Psychiatric disorders			
Anxiety			
subjects affected / exposed	1 / 74 (1.35%)	2 / 83 (2.41%)	1 / 9 (11.11%)
occurrences (all)	1	2	1
Confusional state			
subjects affected / exposed	6 / 74 (8.11%)	6 / 83 (7.23%)	0 / 9 (0.00%)
occurrences (all)	7	7	0
Depressed mood			
subjects affected / exposed	0 / 74 (0.00%)	1 / 83 (1.20%)	1 / 9 (11.11%)
occurrences (all)	0	1	1
Depression			

subjects affected / exposed	1 / 74 (1.35%)	2 / 83 (2.41%)	1 / 9 (11.11%)
occurrences (all)	1	2	1
Insomnia			
subjects affected / exposed	3 / 74 (4.05%)	4 / 83 (4.82%)	1 / 9 (11.11%)
occurrences (all)	3	4	1
Investigations			
Amylase decreased			
subjects affected / exposed	0 / 74 (0.00%)	1 / 83 (1.20%)	1 / 9 (11.11%)
occurrences (all)	0	4	4
Alanine aminotransferase increased			
subjects affected / exposed	12 / 74 (16.22%)	14 / 83 (16.87%)	2 / 9 (22.22%)
occurrences (all)	15	18	3
Aspartate aminotransferase increased			
subjects affected / exposed	4 / 74 (5.41%)	6 / 83 (7.23%)	2 / 9 (22.22%)
occurrences (all)	5	8	3
Blood lactate dehydrogenase increased			
subjects affected / exposed	0 / 74 (0.00%)	1 / 83 (1.20%)	1 / 9 (11.11%)
occurrences (all)	0	1	1
Blood creatinine increased			
subjects affected / exposed	5 / 74 (6.76%)	7 / 83 (8.43%)	2 / 9 (22.22%)
occurrences (all)	5	7	2
Blood creatinine decreased			
subjects affected / exposed	0 / 74 (0.00%)	1 / 83 (1.20%)	1 / 9 (11.11%)
occurrences (all)	0	2	2
Blood alkaline phosphatase increased			
subjects affected / exposed	2 / 74 (2.70%)	3 / 83 (3.61%)	1 / 9 (11.11%)
occurrences (all)	2	3	1
Blood parathyroid hormone decreased			
subjects affected / exposed	1 / 74 (1.35%)	2 / 83 (2.41%)	1 / 9 (11.11%)
occurrences (all)	1	2	1
Blood urea decreased			
subjects affected / exposed	0 / 74 (0.00%)	1 / 83 (1.20%)	1 / 9 (11.11%)
occurrences (all)	0	1	1
Blood phosphorus increased			

subjects affected / exposed	5 / 74 (6.76%)	6 / 83 (7.23%)	1 / 9 (11.11%)
occurrences (all)	5	6	1
Cortisol decreased			
subjects affected / exposed	0 / 74 (0.00%)	1 / 83 (1.20%)	1 / 9 (11.11%)
occurrences (all)	0	1	1
Haemoglobin decreased			
subjects affected / exposed	3 / 74 (4.05%)	4 / 83 (4.82%)	1 / 9 (11.11%)
occurrences (all)	3	4	1
Gamma-glutamyltransferase increased			
subjects affected / exposed	0 / 74 (0.00%)	1 / 83 (1.20%)	1 / 9 (11.11%)
occurrences (all)	0	2	2
Lipase decreased			
subjects affected / exposed	0 / 74 (0.00%)	1 / 83 (1.20%)	1 / 9 (11.11%)
occurrences (all)	0	2	2
Lipase increased			
subjects affected / exposed	5 / 74 (6.76%)	6 / 83 (7.23%)	1 / 9 (11.11%)
occurrences (all)	7	12	5
Lymphocyte count decreased			
subjects affected / exposed	4 / 74 (5.41%)	4 / 83 (4.82%)	0 / 9 (0.00%)
occurrences (all)	5	5	0
Neutrophil count increased			
subjects affected / exposed	0 / 74 (0.00%)	1 / 83 (1.20%)	1 / 9 (11.11%)
occurrences (all)	0	2	2
Transaminases increased			
subjects affected / exposed	0 / 74 (0.00%)	1 / 83 (1.20%)	1 / 9 (11.11%)
occurrences (all)	0	2	2
White blood cell count decreased			
subjects affected / exposed	3 / 74 (4.05%)	4 / 83 (4.82%)	1 / 9 (11.11%)
occurrences (all)	5	6	1
Weight decreased			
subjects affected / exposed	5 / 74 (6.76%)	5 / 83 (6.02%)	0 / 9 (0.00%)
occurrences (all)	5	5	0
Vitamin D decreased			
subjects affected / exposed	4 / 74 (5.41%)	4 / 83 (4.82%)	0 / 9 (0.00%)
occurrences (all)	4	4	0

White blood cell count increased subjects affected / exposed occurrences (all)	1 / 74 (1.35%) 1	2 / 83 (2.41%) 2	1 / 9 (11.11%) 1
Injury, poisoning and procedural complications			
Contusion subjects affected / exposed occurrences (all)	2 / 74 (2.70%) 2	3 / 83 (3.61%) 3	1 / 9 (11.11%) 1
Fall subjects affected / exposed occurrences (all)	6 / 74 (8.11%) 8	6 / 83 (7.23%) 8	0 / 9 (0.00%) 0
Nervous system disorders			
Aphasia subjects affected / exposed occurrences (all)	7 / 74 (9.46%) 7	7 / 83 (8.43%) 7	0 / 9 (0.00%) 0
Dysgeusia subjects affected / exposed occurrences (all)	8 / 74 (10.81%) 8	10 / 83 (12.05%) 10	2 / 9 (22.22%) 2
Headache subjects affected / exposed occurrences (all)	14 / 74 (18.92%) 16	15 / 83 (18.07%) 20	1 / 9 (11.11%) 4
Partial seizures subjects affected / exposed occurrences (all)	1 / 74 (1.35%) 2	2 / 83 (2.41%) 3	1 / 9 (11.11%) 1
Paraesthesia subjects affected / exposed occurrences (all)	5 / 74 (6.76%) 5	6 / 83 (7.23%) 6	1 / 9 (11.11%) 1
Seizure subjects affected / exposed occurrences (all)	10 / 74 (13.51%) 13	11 / 83 (13.25%) 15	1 / 9 (11.11%) 2
Taste disorder subjects affected / exposed occurrences (all)	2 / 74 (2.70%) 2	4 / 83 (4.82%) 4	2 / 9 (22.22%) 2
Blood and lymphatic system disorders			
Anaemia subjects affected / exposed occurrences (all)	2 / 74 (2.70%) 2	3 / 83 (3.61%) 3	1 / 9 (11.11%) 1

Neutropenia subjects affected / exposed occurrences (all)	4 / 74 (5.41%) 4	4 / 83 (4.82%) 4	0 / 9 (0.00%) 0
Eye disorders			
Chalazion subjects affected / exposed occurrences (all)	0 / 74 (0.00%) 0	1 / 83 (1.20%) 2	1 / 9 (11.11%) 2
Dry eye subjects affected / exposed occurrences (all)	8 / 74 (10.81%) 9	11 / 83 (13.25%) 12	3 / 9 (33.33%) 3
Eyelash thickening subjects affected / exposed occurrences (all)	0 / 74 (0.00%) 0	1 / 83 (1.20%) 1	1 / 9 (11.11%) 1
Eye pruritus subjects affected / exposed occurrences (all)	0 / 74 (0.00%) 0	1 / 83 (1.20%) 1	1 / 9 (11.11%) 1
Keratopathy subjects affected / exposed occurrences (all)	0 / 74 (0.00%) 0	1 / 83 (1.20%) 1	1 / 9 (11.11%) 1
Punctate keratitis subjects affected / exposed occurrences (all)	4 / 74 (5.41%) 5	4 / 83 (4.82%) 5	0 / 9 (0.00%) 0
Retinal exudates subjects affected / exposed occurrences (all)	0 / 74 (0.00%) 0	1 / 83 (1.20%) 2	1 / 9 (11.11%) 2
Vision blurred subjects affected / exposed occurrences (all)	5 / 74 (6.76%) 5	6 / 83 (7.23%) 6	1 / 9 (11.11%) 1
Gastrointestinal disorders			
Abdominal pain subjects affected / exposed occurrences (all)	5 / 74 (6.76%) 7	6 / 83 (7.23%) 8	1 / 9 (11.11%) 1
Abdominal discomfort subjects affected / exposed occurrences (all)	0 / 74 (0.00%) 0	1 / 83 (1.20%) 2	1 / 9 (11.11%) 2
Anal haemorrhage			

subjects affected / exposed	0 / 74 (0.00%)	1 / 83 (1.20%)	1 / 9 (11.11%)
occurrences (all)	0	1	1
Constipation			
subjects affected / exposed	16 / 74 (21.62%)	20 / 83 (24.10%)	4 / 9 (44.44%)
occurrences (all)	17	21	4
Dyspepsia			
subjects affected / exposed	1 / 74 (1.35%)	2 / 83 (2.41%)	1 / 9 (11.11%)
occurrences (all)	1	2	1
Diarrhoea			
subjects affected / exposed	33 / 74 (44.59%)	41 / 83 (49.40%)	8 / 9 (88.89%)
occurrences (all)	57	69	12
Dry mouth			
subjects affected / exposed	10 / 74 (13.51%)	13 / 83 (15.66%)	3 / 9 (33.33%)
occurrences (all)	10	14	4
Gastrooesophageal reflux disease			
subjects affected / exposed	2 / 74 (2.70%)	3 / 83 (3.61%)	1 / 9 (11.11%)
occurrences (all)	2	3	1
Gastritis			
subjects affected / exposed	0 / 74 (0.00%)	1 / 83 (1.20%)	1 / 9 (11.11%)
occurrences (all)	0	1	1
Nausea			
subjects affected / exposed	9 / 74 (12.16%)	11 / 83 (13.25%)	2 / 9 (22.22%)
occurrences (all)	9	11	2
Oral pain			
subjects affected / exposed	4 / 74 (5.41%)	4 / 83 (4.82%)	0 / 9 (0.00%)
occurrences (all)	4	4	0
Stomatitis			
subjects affected / exposed	14 / 74 (18.92%)	14 / 83 (16.87%)	0 / 9 (0.00%)
occurrences (all)	21	21	0
Vomiting			
subjects affected / exposed	1 / 74 (1.35%)	2 / 83 (2.41%)	1 / 9 (11.11%)
occurrences (all)	1	2	1
Skin and subcutaneous tissue disorders			
Alopecia			
subjects affected / exposed	16 / 74 (21.62%)	23 / 83 (27.71%)	7 / 9 (77.78%)
occurrences (all)	16	25	9

Dry skin			
subjects affected / exposed	11 / 74 (14.86%)	16 / 83 (19.28%)	5 / 9 (55.56%)
occurrences (all)	14	19	5
Dermatitis atopic			
subjects affected / exposed	0 / 74 (0.00%)	1 / 83 (1.20%)	1 / 9 (11.11%)
occurrences (all)	0	1	1
Hair colour changes			
subjects affected / exposed	2 / 74 (2.70%)	3 / 83 (3.61%)	1 / 9 (11.11%)
occurrences (all)	2	3	1
Nail discolouration			
subjects affected / exposed	3 / 74 (4.05%)	4 / 83 (4.82%)	1 / 9 (11.11%)
occurrences (all)	3	4	1
Madarosis			
subjects affected / exposed	1 / 74 (1.35%)	2 / 83 (2.41%)	1 / 9 (11.11%)
occurrences (all)	1	2	1
Nail disorder			
subjects affected / exposed	15 / 74 (20.27%)	16 / 83 (19.28%)	1 / 9 (11.11%)
occurrences (all)	15	16	1
Onychomadesis			
subjects affected / exposed	4 / 74 (5.41%)	5 / 83 (6.02%)	1 / 9 (11.11%)
occurrences (all)	4	5	1
Onycholysis			
subjects affected / exposed	4 / 74 (5.41%)	8 / 83 (9.64%)	4 / 9 (44.44%)
occurrences (all)	4	8	4
Onychoclasia			
subjects affected / exposed	2 / 74 (2.70%)	3 / 83 (3.61%)	1 / 9 (11.11%)
occurrences (all)	2	3	1
Onychalgia			
subjects affected / exposed	1 / 74 (1.35%)	2 / 83 (2.41%)	1 / 9 (11.11%)
occurrences (all)	1	2	1
Palmar-plantar erythrodysesthesia syndrome			
subjects affected / exposed	5 / 74 (6.76%)	5 / 83 (6.02%)	0 / 9 (0.00%)
occurrences (all)	8	8	0
Endocrine disorders			

Gonadotrophin deficiency subjects affected / exposed occurrences (all)	0 / 74 (0.00%) 0	1 / 83 (1.20%) 1	1 / 9 (11.11%) 1
Hyperthyroidism subjects affected / exposed occurrences (all)	0 / 74 (0.00%) 0	1 / 83 (1.20%) 1	1 / 9 (11.11%) 1
Hypothalamo-pituitary disorder subjects affected / exposed occurrences (all)	0 / 74 (0.00%) 0	1 / 83 (1.20%) 1	1 / 9 (11.11%) 1
Musculoskeletal and connective tissue disorders			
Arthralgia subjects affected / exposed occurrences (all)	6 / 74 (8.11%) 7	7 / 83 (8.43%) 10	1 / 9 (11.11%) 3
Back pain subjects affected / exposed occurrences (all)	1 / 74 (1.35%) 2	3 / 83 (3.61%) 4	2 / 9 (22.22%) 2
Myalgia subjects affected / exposed occurrences (all)	2 / 74 (2.70%) 2	3 / 83 (3.61%) 3	1 / 9 (11.11%) 1
Muscular weakness subjects affected / exposed occurrences (all)	6 / 74 (8.11%) 6	6 / 83 (7.23%) 6	0 / 9 (0.00%) 0
Muscle spasms subjects affected / exposed occurrences (all)	5 / 74 (6.76%) 5	6 / 83 (7.23%) 6	1 / 9 (11.11%) 1
Pain in extremity subjects affected / exposed occurrences (all)	2 / 74 (2.70%) 3	4 / 83 (4.82%) 6	2 / 9 (22.22%) 3
Infections and infestations			
Conjunctivitis subjects affected / exposed occurrences (all)	1 / 74 (1.35%) 1	2 / 83 (2.41%) 2	1 / 9 (11.11%) 1
COVID-19 subjects affected / exposed occurrences (all)	4 / 74 (5.41%) 4	4 / 83 (4.82%) 4	0 / 9 (0.00%) 0
Ear infection			

subjects affected / exposed	0 / 74 (0.00%)	1 / 83 (1.20%)	1 / 9 (11.11%)
occurrences (all)	0	1	1
Nail infection			
subjects affected / exposed	1 / 74 (1.35%)	2 / 83 (2.41%)	1 / 9 (11.11%)
occurrences (all)	1	3	2
Nasopharyngitis			
subjects affected / exposed	1 / 74 (1.35%)	2 / 83 (2.41%)	1 / 9 (11.11%)
occurrences (all)	1	2	1
Pharyngitis			
subjects affected / exposed	0 / 74 (0.00%)	1 / 83 (1.20%)	1 / 9 (11.11%)
occurrences (all)	0	1	1
Paronychia			
subjects affected / exposed	0 / 74 (0.00%)	1 / 83 (1.20%)	1 / 9 (11.11%)
occurrences (all)	0	1	1
Skin infection			
subjects affected / exposed	4 / 74 (5.41%)	4 / 83 (4.82%)	0 / 9 (0.00%)
occurrences (all)	4	4	0
Metabolism and nutrition disorders			
Dehydration			
subjects affected / exposed	4 / 74 (5.41%)	4 / 83 (4.82%)	0 / 9 (0.00%)
occurrences (all)	4	4	0
Decreased appetite			
subjects affected / exposed	5 / 74 (6.76%)	5 / 83 (6.02%)	0 / 9 (0.00%)
occurrences (all)	5	5	0
Hypercalcaemia			
subjects affected / exposed	2 / 74 (2.70%)	4 / 83 (4.82%)	2 / 9 (22.22%)
occurrences (all)	4	8	4
Hyperphosphataemia			
subjects affected / exposed	54 / 74 (72.97%)	61 / 83 (73.49%)	7 / 9 (77.78%)
occurrences (all)	89	100	11
Hypomagnesaemia			
subjects affected / exposed	3 / 74 (4.05%)	4 / 83 (4.82%)	1 / 9 (11.11%)
occurrences (all)	4	5	1
Hyponatraemia			
subjects affected / exposed	5 / 74 (6.76%)	5 / 83 (6.02%)	0 / 9 (0.00%)
occurrences (all)	7	7	0

Hypophosphataemia subjects affected / exposed occurrences (all)	16 / 74 (21.62%) 21	20 / 83 (24.10%) 27	4 / 9 (44.44%) 6
Vitamin D deficiency subjects affected / exposed occurrences (all)	4 / 74 (5.41%) 4	4 / 83 (4.82%) 4	0 / 9 (0.00%) 0

**More information**

**Substantial protocol amendments (globally)**

Were there any global substantial amendments to the protocol? Yes

Date	Amendment
19 November 2021	The primary purpose of this amendment was to incorporate updates based on regulatory agency review of the Protocol.
19 January 2023	The primary purpose of this amendment was to incorporate participants from Cohort C with activating mutations into Cohorts A and B.
26 July 2024	The main purpose of this amendment was to indicate that at the end of the study, participants who continued to receive the study drug would have continued access to it in accordance with local regulations or via Incyte.

Notes:

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**Interruptions (globally)**

Were there any global interruptions to the trial? No

**Limitations and caveats**

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

The study was terminated by the sponsor after the prespecified interim analysis did not meet the futility boundary.
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Notes: