



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 Locally Advanced/Metastatic or Surgically Unresectable Solid Tumor Malignancies Harboring Activating FGFR Mutations or Translocations (FIGHT-207)

Summary

EudraCT number	2018-004768-69
Trial protocol	DK GB DE IT
Global end of trial date	29 March 2022

Results information

Result version number	v1 (current)
This version publication date	04 March 2023
First version publication date	04 March 2023

Trial information

Trial identification

Sponsor protocol code	INCB 54828-207
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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 Drive, Wilmington, United States, 19803
Public contact	Study Director, Incyte Corporation, 1 8554633463, medinfo@incyte.com
Scientific contact	Study Director, Incyte Corporation, 1 8554633463, 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	29 March 2022
Is this the analysis of the primary completion data?	No

Global end of trial reached?	Yes
Global end of trial date	29 March 2022
Was the trial ended prematurely?	Yes

Notes:

General information about the trial

Main objective of the trial:

The purpose of this study was to evaluate the efficacy and safety of pemigatinib in participants with previously treated locally advanced/metastatic or surgically unresectable solid tumor malignancies harboring activating fibroblast growth factor receptor (FGFR) mutations or translocations.

Protection of trial subjects:

This study was to be performed in accordance with ethical principles that have their origin in the Declaration of Helsinki and conducted in adherence to the study Protocol, applicable Good Clinical Practices, and applicable laws and country-specific regulations in which the study was being conducted.

Background therapy: -

Evidence for comparator: -

Actual start date of recruitment	17 October 2019
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: 6
Country: Number of subjects enrolled	France: 16
Country: Number of subjects enrolled	Germany: 1
Country: Number of subjects enrolled	Israel: 2
Country: Number of subjects enrolled	Italy: 9
Country: Number of subjects enrolled	Japan: 21
Country: Number of subjects enrolled	Korea, Republic of: 4
Country: Number of subjects enrolled	Spain: 17
Country: Number of subjects enrolled	United Kingdom: 3
Country: Number of subjects enrolled	United States: 32
Worldwide total number of subjects	111
EEA total number of subjects	49

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)	66
From 65 to 84 years	45
85 years and over	0

Subject disposition

Recruitment

Recruitment details: -

Pre-assignment

Screening details:

This study enrolled participants at 49 study sites in the United States, South Korea, United Kingdom, France, Italy, Israel, Germany, Spain, Denmark, and Japan.

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
Arm title	Cohort A: FGFR1-3 in-frame fusions or FGFR2 arrangements

Arm description:

Participants with fibroblast growth factor receptor (FGFR) 1-3 in-frame fusions or fibroblast growth factor receptor 2 (FGFR2) rearrangements self-administered oral pemigatinib at a starting dose of 13.5 milligrams (mg) once daily (QD) continuously in 21-day cycles. Pemigatinib was administered until documented disease progression or unacceptable toxicity.

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:

4.5-, 9-, and 13.5-mg tablets; starting dose of 13.5 mg

Arm title	Cohort B: known or likely activating FGFR1-3 mutations
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Arm description:

Participants with known or likely activating mutations in FGFR1-3 self-administered oral pemigatinib at a starting dose of 13.5 mg QD continuously in 21-day cycles. Pemigatinib was administered until documented disease progression or unacceptable toxicity.

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:

4.5-, 9-, and 13.5-mg tablets; starting dose of 13.5 mg

Arm title	Cohort C: other FGFR mutations or arrangements
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Arm description:

Participants with other FGFR mutations or arrangements self-administered oral pemigatinib at a starting dose of 13.5 mg QD continuously in 21-day cycles. Pemigatinib was administered until documented disease progression or unacceptable toxicity.

Arm type	Experimental
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Investigational medicinal product name	pemigatinib
Investigational medicinal product code	
Other name	
Pharmaceutical forms	Tablet
Routes of administration	Oral use
Dosage and administration details: 4.5-, 9-, and 13.5-mg tablets; starting dose of 13.5 mg	
Arm title	Other

Arm description:

Participants with an FGF/FGFR status for whom the local laboratory FGF/FGFR results could not be confirmed centrally self-administered oral pemigatinib at a starting dose of 13.5 mg QD continuously in 21-day cycles. Pemigatinib was administered until documented disease progression or unacceptable toxicity.

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:

4.5-, 9-, and 13.5-mg tablets; starting dose of 13.5 mg

Number of subjects in period 1	Cohort A: FGFR1-3 in-frame fusions or FGFR2 arrangements	Cohort B: known or likely activating FGFR1-3 mutations	Cohort C: other FGFR mutations or arrangements
Started	49	32	26
Completed	0	0	0
Not completed	49	32	26
Adverse event, serious fatal	18	17	12
Consent withdrawn by subject	4	-	3
Never Returned to Hospital	-	-	1
Study Terminated by Sponsor	25	11	6
Lost to follow-up	1	2	3
Disease Progression	1	2	1

Number of subjects in period 1	Other
Started	4
Completed	0
Not completed	4
Adverse event, serious fatal	4
Consent withdrawn by subject	-
Never Returned to Hospital	-
Study Terminated by Sponsor	-
Lost to follow-up	-

Disease Progression	-
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Baseline characteristics

Reporting groups

Reporting group title	Cohort A: FGFR1-3 in-frame fusions or FGFR2 arrangements
Reporting group description: Participants with fibroblast growth factor receptor (FGFR) 1-3 in-frame fusions or fibroblast growth factor receptor 2 (FGFR2) rearrangements self-administered oral pemigatinib at a starting dose of 13.5 milligrams (mg) once daily (QD) continuously in 21-day cycles. Pemigatinib was administered until documented disease progression or unacceptable toxicity.	
Reporting group title	Cohort B: known or likely activating FGFR1-3 mutations
Reporting group description: Participants with known or likely activating mutations in FGFR1-3 self-administered oral pemigatinib at a starting dose of 13.5 mg QD continuously in 21-day cycles. Pemigatinib was administered until documented disease progression or unacceptable toxicity.	
Reporting group title	Cohort C: other FGFR mutations or arrangements
Reporting group description: Participants with other FGFR mutations or arrangements self-administered oral pemigatinib at a starting dose of 13.5 mg QD continuously in 21-day cycles. Pemigatinib was administered until documented disease progression or unacceptable toxicity.	
Reporting group title	Other
Reporting group description: Participants with an FGF/FGFR status for whom the local laboratory FGF/FGFR results could not be confirmed centrally self-administered oral pemigatinib at a starting dose of 13.5 mg QD continuously in 21-day cycles. Pemigatinib was administered until documented disease progression or unacceptable toxicity.	

Reporting group values	Cohort A: FGFR1-3 in-frame fusions or FGFR2 arrangements	Cohort B: known or likely activating FGFR1-3 mutations	Cohort C: other FGFR mutations or arrangements
Number of subjects	49	32	26
Age categorical Units: Subjects			
Adults (18-64 years)	34	12	17
From 65-84 years	15	20	9
85 years and over	0	0	0
Age Continuous Units: years			
arithmetic mean	59.4	66.0	61.5
standard deviation	± 11.51	± 10.04	± 13.32
Sex: Female, Male Units: participants			
Female	28	19	14
Male	21	13	12
Race, Customized Units: Subjects			
White	38	20	16
Black or African American	0	0	1
Asian	9	9	7
Participant Declined to Provide	1	0	0
Not Available	0	1	0
Not Required in Country of Origin	0	1	0
Missing	1	1	2

Ethnicity, Customized			
Units: Subjects			
Hispanic or Latino	1	1	1
Not Hispanic or Latino	44	25	20
Not Reported	2	2	3
Unknown	1	2	0
Captured as "Other"	0	1	0
Missing	1	1	2

Reporting group values	Other	Total	
Number of subjects	4	111	
Age categorical			
Units: Subjects			
Adults (18-64 years)	3	66	
From 65-84 years	1	45	
85 years and over	0	0	
Age Continuous			
Units: years			
arithmetic mean	48.0		
standard deviation	± 14.45	-	
Sex: Female, Male			
Units: participants			
Female	1	62	
Male	3	49	
Race, Customized			
Units: Subjects			
White	0	74	
Black or African American	0	1	
Asian	4	29	
Participant Declined to Provide	0	1	
Not Available	0	1	
Not Required in Country of Origin	0	1	
Missing	0	4	
Ethnicity, Customized			
Units: Subjects			
Hispanic or Latino	0	3	
Not Hispanic or Latino	3	92	
Not Reported	1	8	
Unknown	0	3	
Captured as "Other"	0	1	
Missing	0	4	

End points

End points reporting groups

Reporting group title	Cohort A: FGFR1-3 in-frame fusions or FGFR2 arrangements
Reporting group description: Participants with fibroblast growth factor receptor (FGFR) 1-3 in-frame fusions or fibroblast growth factor receptor 2 (FGFR2) rearrangements self-administered oral pemigatinib at a starting dose of 13.5 milligrams (mg) once daily (QD) continuously in 21-day cycles. Pemigatinib was administered until documented disease progression or unacceptable toxicity.	
Reporting group title	Cohort B: known or likely activating FGFR1-3 mutations
Reporting group description: Participants with known or likely activating mutations in FGFR1-3 self-administered oral pemigatinib at a starting dose of 13.5 mg QD continuously in 21-day cycles. Pemigatinib was administered until documented disease progression or unacceptable toxicity.	
Reporting group title	Cohort C: other FGFR mutations or arrangements
Reporting group description: Participants with other FGFR mutations or arrangements self-administered oral pemigatinib at a starting dose of 13.5 mg QD continuously in 21-day cycles. Pemigatinib was administered until documented disease progression or unacceptable toxicity.	
Reporting group title	Other
Reporting group description: Participants with an FGF/FGFR status for whom the local laboratory FGF/FGFR results could not be confirmed centrally self-administered oral pemigatinib at a starting dose of 13.5 mg QD continuously in 21-day cycles. Pemigatinib was administered until documented disease progression or unacceptable toxicity.	

Primary: Objective Response Rate (ORR), defined as the percentage of participants with a best overall response of complete response (CR) or partial response (PR) based on RECIST v1.1 or RANO, in participants with FGFR1-3 in-frame fusions or FGFR2 arrangements

End point title	Objective Response Rate (ORR), defined as the percentage of participants with a best overall response of complete response (CR) or partial response (PR) based on RECIST v1.1 or RANO, in participants with FGFR1-3 in-frame fusions or FGFR2 arrangements ^[1]
End point description: Per Response Evaluation Criteria in Solid Tumors version 1.1 (RECIST v1.1): CR: disappearance of all target/non-target lesions; no appearance of new lesions. PR: complete disappearance or a $\geq 30\%$ decrease in the sum of the diameters of target lesions, taking as a reference the baseline sum diameters; no new lesions; no progression of non-target lesions. Per Response Assessment in Neuro-Oncology (RANO; for participants with primary brain tumors): CR: disappearance of all enhancing lesions; stable/improved non-enhancing lesions; stable/improved clinically. PR: $\geq 50\%$ decrease in sum of perpendicular diameters of measurable enhancing lesions; no progression of non-measurable disease; stable/improved non-enhancing lesions; stable/improved clinically. Cohort determination was based on FGFR status from a central genomics laboratory. Response data were from an independent centralized radiological review committee per RECIST v1.1 and RANO, and response was confirmed.	
End point type	Primary
End point timeframe: up to 483 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	Cohort A: FGFR1-3 in-frame fusions or FGFR2 arrangements	Cohort B: known or likely activating FGFR1-3 mutations	Cohort C: other FGFR mutations or arrangements	Other
Subject group type	Reporting group	Reporting group	Reporting group	Reporting group
Number of subjects analysed	49	0 ^[2]	0 ^[3]	0 ^[4]
Units: percentage of participants				
number (confidence interval 95%)	26.5 (14.95 to 41.08)	(to)	(to)	(to)

Notes:

[2] - Analysis was conducted in participants with FGFR1-3 in-frame fusions or FGFR2 arrangements.

[3] - Analysis was conducted in participants with FGFR1-3 in-frame fusions or FGFR2 arrangements.

[4] - Analysis was conducted in participants with FGFR1-3 in-frame fusions or FGFR2 arrangements.

Statistical analyses

No statistical analyses for this end point

Primary: ORR, defined as the percentage of participants with a best overall response of CR or PR based on RECIST v1.1 or RANO, in participants with known or likely activating FGFR1-3 mutations

End point title	ORR, defined as the percentage of participants with a best overall response of CR or PR based on RECIST v1.1 or RANO, in participants with known or likely activating FGFR1-3 mutations ^[5]
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End point description:

Per RECIST v1.1: CR: disappearance of all target/non-target lesions; no appearance of new lesions. PR: complete disappearance or a $\geq 30\%$ decrease in the sum of the diameters of target lesions, taking as a reference the baseline sum diameters; no new lesions; no progression of non-target lesions. Per RANO (for participants with primary brain tumors): CR: disappearance of all enhancing lesions; stable/improved non-enhancing lesions; stable/improved clinically. PR: $\geq 50\%$ decrease in sum of perpendicular diameters of measurable enhancing lesions; no progression of non-measurable disease; stable/improved non-enhancing lesions; stable/improved clinically. Cohort determination was based on FGFR status from a central genomics laboratory. Response data were from an independent centralized radiological review committee per RECIST v1.1 and RANO, and response was confirmed.

End point type	Primary
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End point timeframe:

up to 449 days

Notes:

[5] - 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	Cohort A: FGFR1-3 in-frame fusions or FGFR2 arrangements	Cohort B: known or likely activating FGFR1-3 mutations	Cohort C: other FGFR mutations or arrangements	Other
Subject group type	Reporting group	Reporting group	Reporting group	Reporting group
Number of subjects analysed	0 ^[6]	32	0 ^[7]	0 ^[8]
Units: percentage of participants				
number (confidence interval 95%)	(to)	9.4 (1.98 to 25.02)	(to)	(to)

Notes:

[6] - Analysis was conducted in participants with known or likely activating FGFR1-3 in-frame mutations.

[7] - Analysis was conducted in participants with known or likely activating FGFR1-3 in-frame mutations.

[8] - Analysis was conducted in participants with known or likely activating FGFR1-3 in-frame mutations.

Statistical analyses

No statistical analyses for this end point

Secondary: Progression-free survival (PFS) in participants with FGFR1-3 in-frame fusions or FGFR2 arrangements and in participants with known or likely activating FGFR1-3 mutations

End point title	Progression-free survival (PFS) in participants with FGFR1-3 in-frame fusions or FGFR2 arrangements and in participants with known or likely activating FGFR1-3 mutations
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End point description:

PFS was defined as the time from the first dose until progressive disease (according to RECIST v1.1 or RANO for participants with primary brain tumors and assessed by an independent centralized radiological review committee) or death (whichever occurred first).

End point type	Secondary
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End point timeframe:

up to 532 days

End point values	Cohort A: FGFR1-3 in-frame fusions or FGFR2 arrangements	Cohort B: known or likely activating FGFR1-3 mutations	Cohort C: other FGFR mutations or arrangements	Other
Subject group type	Reporting group	Reporting group	Reporting group	Reporting group
Number of subjects analysed	49	32	0 ^[9]	0 ^[10]
Units: months				
median (confidence interval 95%)	4.53 (3.58 to 6.28)	3.68 (2.07 to 4.47)	(to)	(to)

Notes:

[9] - Analysis was conducted in participants in Cohorts A and B.

[10] - Analysis was conducted in participants in Cohorts A and B.

Statistical analyses

No statistical analyses for this end point

Secondary: Duration of response (DOR), defined as the first CR or PR assessment until progressive disease (PD) or death, in participants with FGFR1-3 in-frame fusions or FGFR2 arrangements and in participants with known or likely activating FGFR1-3 mutations

End point title	Duration of response (DOR), defined as the first CR or PR assessment until progressive disease (PD) or death, in participants with FGFR1-3 in-frame fusions or FGFR2 arrangements and in participants with known or likely activating FGFR1-3 mutations
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End point description:

Assessment was by an independent centralized radiological review committee; response was confirmed. Per RECIST v1.1: CR: disappearance of all target (TLs)/non-target lesions (NTLs); no appearance of new lesions. PR: complete disappearance or a $\geq 30\%$ decrease in the sum of the diameters of TLs, taking as a reference the baseline sum diameters; no new lesions; no progression of NTLs. PD: progression of a TL/NTL or presence of new lesion. Per RANO (participants with primary brain tumors): CR: disappearance of all enhancing lesions (ELs); stable/improved non-enhancing lesions (NELs); stable/improved clinically. PR: $\geq 50\%$ decrease in sum of perpendicular diameters of measurable ELs; no progression of non-measurable disease; stable/improved NELs; stable/improved clinically. PD: $>25\%$ increase in sum of perpendicular diameters of all measurable ELs; significant increase of NELs; new lesions; clear clinical deterioration; failure to return for evaluation due to death/deteriorating condition.

End point type	Secondary
End point timeframe: up to 24.90 months	

End point values	Cohort A: FGFR1-3 in-frame fusions or FGFR2 arrangements	Cohort B: known or likely activating FGFR1-3 mutations	Cohort C: other FGFR mutations or arrangements	Other
Subject group type	Reporting group	Reporting group	Reporting group	Reporting group
Number of subjects analysed	13 ^[11]	3 ^[12]	0 ^[13]	0 ^[14]
Units: months				
median (confidence interval 95%)	7.79 (4.17 to 9999)	6.93 (4.01 to 9999)	(to)	(to)

Notes:

[11] - 9999=not estimable; too few participants had PD/died. Participants with CR or PR were analyzed.

[12] - 9999=not estimable; too few participants had PD/died. Participants with CR or PR were analyzed.

[13] - Analysis was conducted in participants in Cohorts A and B.

[14] - Analysis was conducted in participants in Cohorts A and B.

Statistical analyses

No statistical analyses for this end point

Secondary: Overall survival in participants with FGFR1-3 in-frame fusions or FGFR2 arrangements and in participants with known or likely activating FGFR1-3 mutations

End point title	Overall survival in participants with FGFR1-3 in-frame fusions or FGFR2 arrangements and in participants with known or likely activating FGFR1-3 mutations
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End point description:

Overall survival was defined as the time from the first dose of study drug to death of any cause.
9999=the upper limit of the confidence interval was not estimable because too few participants had died.

End point type	Secondary
End point timeframe: up to 532 days	

End point values	Cohort A: FGFR1-3 in-frame fusions or FGFR2 arrangements	Cohort B: known or likely activating FGFR1-3 mutations	Cohort C: other FGFR mutations or arrangements	Other
Subject group type	Reporting group	Reporting group	Reporting group	Reporting group
Number of subjects analysed	49	32	0 ^[15]	0 ^[16]
Units: months				
median (confidence interval 95%)	17.48 (7.79 to 9999)	11.37 (6.57 to 9999)	(to)	(to)

Notes:

[15] - Analysis was conducted in participants in Cohorts A and B.

[16] - Analysis was conducted in participants in Cohorts A and B.

Statistical analyses

No statistical analyses for this end point

Secondary: Number of participants with any treatment-emergent adverse event (TEAE) and any treatment-related adverse event (AE)

End point title	Number of participants with any treatment-emergent adverse event (TEAE) and any treatment-related adverse event (AE)
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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 considered drug-related. An AE could be any unfavorable and unintended sign (including an abnormal laboratory finding), symptom, or disease (new or exacerbated) temporally associated with the use of study treatment. TEAEs were defined as AEs reported for the first time or the worsening of pre-existing events after the first dose of study treatment. Treatment-related AEs were defined as TEAEs judged as related by the investigator or with a missing causality.

End point type	Secondary
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End point timeframe:

up to 651 days

End point values	Cohort A: FGFR1-3 in- frame fusions or FGFR2 arrangements	Cohort B: known or likely activating FGFR1-3 mutations	Cohort C: other FGFR mutations or arrangements	Other
Subject group type	Reporting group	Reporting group	Reporting group	Reporting group
Number of subjects analysed	49	32	26	4
Units: participants				
TEAEs	49	32	26	4
Treatment-related AEs	46	32	26	4

Statistical analyses

No statistical analyses for this end point

Adverse events

Adverse events information

Timeframe for reporting adverse events:

up to 651 days

Adverse event reporting additional description:

Treatment-emergent adverse events (TEAEs), defined as adverse events reported for the first time or the worsening of pre-existing events after the first dose of study treatment, are reported.

Assessment type	Systematic
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Dictionary used

Dictionary name	MedDRA
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Dictionary version	24.0
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Reporting groups

Reporting group title	Cohort A: FGFR1-3 in-frame fusions or FGFR2 arrangements
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Reporting group description:

Participants with fibroblast growth factor receptor (FGFR) 1-3 in-frame fusions or fibroblast growth factor receptor 2 (FGFR2) rearrangements self-administered oral pemigatinib at a starting dose of 13.5 milligrams (mg) once daily (QD) continuously in 21-day cycles. Pemigatinib was administered until documented disease progression or unacceptable toxicity.

Reporting group title	Cohort B: known or likely activating FGFR1-3 mutations
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Reporting group description:

Participants with known or likely activating mutations in FGFR1-3 self-administered oral pemigatinib at a starting dose of 13.5 mg QD continuously in 21-day cycles. Pemigatinib was administered until documented disease progression or unacceptable toxicity.

Reporting group title	Total
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Reporting group description:

Total

Reporting group title	Other
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Reporting group description:

Participants with an FGF/FGFR status for whom the local laboratory FGF/FGFR results could not be confirmed centrally self-administered oral pemigatinib at a starting dose of 13.5 mg QD continuously in 21-day cycles. Pemigatinib was administered until documented disease progression or unacceptable toxicity.

Reporting group title	Cohort C: other FGFR mutations or arrangements
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Reporting group description:

Participants with other FGFR mutations or arrangements self-administered oral pemigatinib at a starting dose of 13.5 mg QD continuously in 21-day cycles. Pemigatinib was administered until documented disease progression or unacceptable toxicity.

Serious adverse events	Cohort A: FGFR1-3 in-frame fusions or FGFR2 arrangements	Cohort B: known or likely activating FGFR1-3 mutations	Total
Total subjects affected by serious adverse events			
subjects affected / exposed	21 / 49 (42.86%)	7 / 32 (21.88%)	40 / 111 (36.04%)
number of deaths (all causes)	18	17	52
number of deaths resulting from adverse events	2	2	6
Neoplasms benign, malignant and unspecified (incl cysts and polyps)			
Metastases to central nervous system			

subjects affected / exposed	1 / 49 (2.04%)	0 / 32 (0.00%)	1 / 111 (0.90%)
occurrences causally related to treatment / all	0 / 1	0 / 0	0 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
General disorders and administration site conditions			
Fatigue			
subjects affected / exposed	0 / 49 (0.00%)	0 / 32 (0.00%)	1 / 111 (0.90%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 2
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
General physical health deterioration			
subjects affected / exposed	0 / 49 (0.00%)	1 / 32 (3.13%)	3 / 111 (2.70%)
occurrences causally related to treatment / all	0 / 0	0 / 1	0 / 3
deaths causally related to treatment / all	0 / 0	0 / 1	0 / 3
Oedema peripheral			
subjects affected / exposed	1 / 49 (2.04%)	0 / 32 (0.00%)	1 / 111 (0.90%)
occurrences causally related to treatment / all	1 / 1	0 / 0	1 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Respiratory, thoracic and mediastinal disorders			
Acute respiratory failure			
subjects affected / exposed	0 / 49 (0.00%)	1 / 32 (3.13%)	1 / 111 (0.90%)
occurrences causally related to treatment / all	0 / 0	0 / 1	0 / 1
deaths causally related to treatment / all	0 / 0	0 / 1	0 / 1
Pneumothorax			
subjects affected / exposed	1 / 49 (2.04%)	0 / 32 (0.00%)	1 / 111 (0.90%)
occurrences causally related to treatment / all	0 / 1	0 / 0	0 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Pulmonary embolism			
subjects affected / exposed	1 / 49 (2.04%)	0 / 32 (0.00%)	1 / 111 (0.90%)
occurrences causally related to treatment / all	0 / 1	0 / 0	0 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Psychiatric disorders			
Confusional state			

subjects affected / exposed	2 / 49 (4.08%)	0 / 32 (0.00%)	2 / 111 (1.80%)
occurrences causally related to treatment / all	0 / 2	0 / 0	0 / 2
deaths causally related to treatment / all	0 / 1	0 / 0	0 / 1
Product issues			
Stent malfunction			
subjects affected / exposed	1 / 49 (2.04%)	0 / 32 (0.00%)	1 / 111 (0.90%)
occurrences causally related to treatment / all	0 / 1	0 / 0	0 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Investigations			
Blood creatinine increased			
subjects affected / exposed	0 / 49 (0.00%)	1 / 32 (3.13%)	1 / 111 (0.90%)
occurrences causally related to treatment / all	0 / 0	0 / 1	0 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Blood bilirubin increased			
subjects affected / exposed	1 / 49 (2.04%)	0 / 32 (0.00%)	2 / 111 (1.80%)
occurrences causally related to treatment / all	0 / 1	0 / 0	0 / 2
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Injury, poisoning and procedural complications			
Post procedural haematuria			
subjects affected / exposed	0 / 49 (0.00%)	0 / 32 (0.00%)	1 / 111 (0.90%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Cardiac disorders			
Long QT syndrome			
subjects affected / exposed	0 / 49 (0.00%)	0 / 32 (0.00%)	1 / 111 (0.90%)
occurrences causally related to treatment / all	0 / 0	0 / 0	1 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Nervous system disorders			
Headache			
subjects affected / exposed	1 / 49 (2.04%)	0 / 32 (0.00%)	1 / 111 (0.90%)
occurrences causally related to treatment / all	0 / 1	0 / 0	0 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Spinal cord compression			

subjects affected / exposed	1 / 49 (2.04%)	0 / 32 (0.00%)	1 / 111 (0.90%)
occurrences causally related to treatment / all	0 / 1	0 / 0	0 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Blood and lymphatic system disorders			
Febrile neutropenia			
subjects affected / exposed	1 / 49 (2.04%)	0 / 32 (0.00%)	1 / 111 (0.90%)
occurrences causally related to treatment / all	1 / 1	0 / 0	1 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Lymphopenia			
subjects affected / exposed	0 / 49 (0.00%)	0 / 32 (0.00%)	1 / 111 (0.90%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Eye disorders			
Corneal erosion			
subjects affected / exposed	1 / 49 (2.04%)	0 / 32 (0.00%)	1 / 111 (0.90%)
occurrences causally related to treatment / all	1 / 1	0 / 0	1 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Gastrointestinal disorders			
Abdominal pain			
subjects affected / exposed	3 / 49 (6.12%)	0 / 32 (0.00%)	3 / 111 (2.70%)
occurrences causally related to treatment / all	0 / 3	0 / 0	0 / 3
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Ascites			
subjects affected / exposed	1 / 49 (2.04%)	0 / 32 (0.00%)	1 / 111 (0.90%)
occurrences causally related to treatment / all	0 / 1	0 / 0	0 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Constipation			
subjects affected / exposed	1 / 49 (2.04%)	0 / 32 (0.00%)	1 / 111 (0.90%)
occurrences causally related to treatment / all	0 / 1	0 / 0	0 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Haematemesis			
subjects affected / exposed	2 / 49 (4.08%)	0 / 32 (0.00%)	2 / 111 (1.80%)
occurrences causally related to treatment / all	0 / 2	0 / 0	0 / 2
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0

Intestinal obstruction			
subjects affected / exposed	1 / 49 (2.04%)	0 / 32 (0.00%)	1 / 111 (0.90%)
occurrences causally related to treatment / all	0 / 1	0 / 0	0 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Nausea			
subjects affected / exposed	0 / 49 (0.00%)	0 / 32 (0.00%)	1 / 111 (0.90%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Obstruction gastric			
subjects affected / exposed	1 / 49 (2.04%)	0 / 32 (0.00%)	1 / 111 (0.90%)
occurrences causally related to treatment / all	0 / 1	0 / 0	0 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Pancreatitis			
subjects affected / exposed	1 / 49 (2.04%)	0 / 32 (0.00%)	1 / 111 (0.90%)
occurrences causally related to treatment / all	1 / 1	0 / 0	1 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Vomiting			
subjects affected / exposed	0 / 49 (0.00%)	0 / 32 (0.00%)	2 / 111 (1.80%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 2
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Hepatobiliary disorders			
Biliary obstruction			
subjects affected / exposed	1 / 49 (2.04%)	0 / 32 (0.00%)	1 / 111 (0.90%)
occurrences causally related to treatment / all	0 / 1	0 / 0	0 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Bile duct stenosis			
subjects affected / exposed	1 / 49 (2.04%)	0 / 32 (0.00%)	1 / 111 (0.90%)
occurrences causally related to treatment / all	0 / 1	0 / 0	0 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Cholangitis			
subjects affected / exposed	0 / 49 (0.00%)	0 / 32 (0.00%)	1 / 111 (0.90%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Skin and subcutaneous tissue disorders			

Cutaneous calcification			
subjects affected / exposed	1 / 49 (2.04%)	0 / 32 (0.00%)	1 / 111 (0.90%)
occurrences causally related to treatment / all	1 / 1	0 / 0	1 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Stevens-Johnson syndrome			
subjects affected / exposed	1 / 49 (2.04%)	0 / 32 (0.00%)	1 / 111 (0.90%)
occurrences causally related to treatment / all	1 / 1	0 / 0	1 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Renal and urinary disorders			
Acute kidney injury			
subjects affected / exposed	0 / 49 (0.00%)	1 / 32 (3.13%)	1 / 111 (0.90%)
occurrences causally related to treatment / all	0 / 0	0 / 1	0 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Hydronephrosis			
subjects affected / exposed	1 / 49 (2.04%)	0 / 32 (0.00%)	1 / 111 (0.90%)
occurrences causally related to treatment / all	0 / 1	0 / 0	0 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Ureteric obstruction			
subjects affected / exposed	0 / 49 (0.00%)	0 / 32 (0.00%)	1 / 111 (0.90%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Urinary tract obstruction			
subjects affected / exposed	1 / 49 (2.04%)	0 / 32 (0.00%)	1 / 111 (0.90%)
occurrences causally related to treatment / all	0 / 1	0 / 0	0 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Musculoskeletal and connective tissue disorders			
Back pain			
subjects affected / exposed	0 / 49 (0.00%)	0 / 32 (0.00%)	1 / 111 (0.90%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Pathological fracture			
subjects affected / exposed	1 / 49 (2.04%)	0 / 32 (0.00%)	1 / 111 (0.90%)
occurrences causally related to treatment / all	0 / 1	0 / 0	0 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0

Rhabdomyolysis			
subjects affected / exposed	0 / 49 (0.00%)	0 / 32 (0.00%)	1 / 111 (0.90%)
occurrences causally related to treatment / all	0 / 0	0 / 0	1 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Infections and infestations			
Cellulitis			
subjects affected / exposed	1 / 49 (2.04%)	0 / 32 (0.00%)	2 / 111 (1.80%)
occurrences causally related to treatment / all	0 / 2	0 / 0	0 / 3
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Fascial infection			
subjects affected / exposed	0 / 49 (0.00%)	0 / 32 (0.00%)	1 / 111 (0.90%)
occurrences causally related to treatment / all	0 / 0	0 / 0	1 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Implant site infection			
subjects affected / exposed	0 / 49 (0.00%)	1 / 32 (3.13%)	1 / 111 (0.90%)
occurrences causally related to treatment / all	0 / 0	0 / 1	0 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Pneumonia			
subjects affected / exposed	1 / 49 (2.04%)	0 / 32 (0.00%)	1 / 111 (0.90%)
occurrences causally related to treatment / all	2 / 3	0 / 0	2 / 3
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Sepsis			
subjects affected / exposed	2 / 49 (4.08%)	0 / 32 (0.00%)	2 / 111 (1.80%)
occurrences causally related to treatment / all	0 / 2	0 / 0	0 / 2
deaths causally related to treatment / all	0 / 1	0 / 0	0 / 1
Pyelonephritis acute			
subjects affected / exposed	0 / 49 (0.00%)	1 / 32 (3.13%)	1 / 111 (0.90%)
occurrences causally related to treatment / all	0 / 0	0 / 1	0 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Urinary tract infection			
subjects affected / exposed	1 / 49 (2.04%)	1 / 32 (3.13%)	2 / 111 (1.80%)
occurrences causally related to treatment / all	0 / 1	0 / 1	0 / 2
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Metabolism and nutrition disorders			

Decreased appetite			
subjects affected / exposed	0 / 49 (0.00%)	0 / 32 (0.00%)	1 / 111 (0.90%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Dehydration			
subjects affected / exposed	0 / 49 (0.00%)	0 / 32 (0.00%)	1 / 111 (0.90%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Hypercalcaemia			
subjects affected / exposed	0 / 49 (0.00%)	0 / 32 (0.00%)	2 / 111 (1.80%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 2
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Hyponatraemia			
subjects affected / exposed	1 / 49 (2.04%)	0 / 32 (0.00%)	1 / 111 (0.90%)
occurrences causally related to treatment / all	1 / 1	0 / 0	1 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0

Serious adverse events	Other	Cohort C: other FGFR mutations or arrangements	
Total subjects affected by serious adverse events			
subjects affected / exposed	2 / 4 (50.00%)	10 / 26 (38.46%)	
number of deaths (all causes)	4	13	
number of deaths resulting from adverse events	0	2	
Neoplasms benign, malignant and unspecified (incl cysts and polyps)			
Metastases to central nervous system			
subjects affected / exposed	0 / 4 (0.00%)	0 / 26 (0.00%)	
occurrences causally related to treatment / all	0 / 0	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
General disorders and administration site conditions			
Fatigue			
subjects affected / exposed	0 / 4 (0.00%)	1 / 26 (3.85%)	
occurrences causally related to treatment / all	0 / 0	0 / 2	
deaths causally related to treatment / all	0 / 0	0 / 0	
General physical health deterioration			

subjects affected / exposed	0 / 4 (0.00%)	2 / 26 (7.69%)	
occurrences causally related to treatment / all	0 / 0	0 / 2	
deaths causally related to treatment / all	0 / 0	0 / 2	
Oedema peripheral			
subjects affected / exposed	0 / 4 (0.00%)	0 / 26 (0.00%)	
occurrences causally related to treatment / all	0 / 0	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Respiratory, thoracic and mediastinal disorders			
Acute respiratory failure			
subjects affected / exposed	0 / 4 (0.00%)	0 / 26 (0.00%)	
occurrences causally related to treatment / all	0 / 0	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Pneumothorax			
subjects affected / exposed	0 / 4 (0.00%)	0 / 26 (0.00%)	
occurrences causally related to treatment / all	0 / 0	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Pulmonary embolism			
subjects affected / exposed	0 / 4 (0.00%)	0 / 26 (0.00%)	
occurrences causally related to treatment / all	0 / 0	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Psychiatric disorders			
Confusional state			
subjects affected / exposed	0 / 4 (0.00%)	0 / 26 (0.00%)	
occurrences causally related to treatment / all	0 / 0	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Product issues			
Stent malfunction			
subjects affected / exposed	0 / 4 (0.00%)	0 / 26 (0.00%)	
occurrences causally related to treatment / all	0 / 0	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Investigations			
Blood creatinine increased			

subjects affected / exposed	0 / 4 (0.00%)	0 / 26 (0.00%)	
occurrences causally related to treatment / all	0 / 0	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Blood bilirubin increased			
subjects affected / exposed	0 / 4 (0.00%)	1 / 26 (3.85%)	
occurrences causally related to treatment / all	0 / 0	0 / 1	
deaths causally related to treatment / all	0 / 0	0 / 0	
Injury, poisoning and procedural complications			
Post procedural haematuria			
subjects affected / exposed	0 / 4 (0.00%)	1 / 26 (3.85%)	
occurrences causally related to treatment / all	0 / 0	0 / 1	
deaths causally related to treatment / all	0 / 0	0 / 0	
Cardiac disorders			
Long QT syndrome			
subjects affected / exposed	0 / 4 (0.00%)	1 / 26 (3.85%)	
occurrences causally related to treatment / all	0 / 0	1 / 1	
deaths causally related to treatment / all	0 / 0	0 / 0	
Nervous system disorders			
Headache			
subjects affected / exposed	0 / 4 (0.00%)	0 / 26 (0.00%)	
occurrences causally related to treatment / all	0 / 0	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Spinal cord compression			
subjects affected / exposed	0 / 4 (0.00%)	0 / 26 (0.00%)	
occurrences causally related to treatment / all	0 / 0	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Blood and lymphatic system disorders			
Febrile neutropenia			
subjects affected / exposed	0 / 4 (0.00%)	0 / 26 (0.00%)	
occurrences causally related to treatment / all	0 / 0	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Lymphopenia			

subjects affected / exposed	1 / 4 (25.00%)	0 / 26 (0.00%)	
occurrences causally related to treatment / all	0 / 1	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Eye disorders			
Corneal erosion			
subjects affected / exposed	0 / 4 (0.00%)	0 / 26 (0.00%)	
occurrences causally related to treatment / all	0 / 0	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Gastrointestinal disorders			
Abdominal pain			
subjects affected / exposed	0 / 4 (0.00%)	0 / 26 (0.00%)	
occurrences causally related to treatment / all	0 / 0	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Ascites			
subjects affected / exposed	0 / 4 (0.00%)	0 / 26 (0.00%)	
occurrences causally related to treatment / all	0 / 0	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Constipation			
subjects affected / exposed	0 / 4 (0.00%)	0 / 26 (0.00%)	
occurrences causally related to treatment / all	0 / 0	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Haematemesis			
subjects affected / exposed	0 / 4 (0.00%)	0 / 26 (0.00%)	
occurrences causally related to treatment / all	0 / 0	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Intestinal obstruction			
subjects affected / exposed	0 / 4 (0.00%)	0 / 26 (0.00%)	
occurrences causally related to treatment / all	0 / 0	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Nausea			
subjects affected / exposed	0 / 4 (0.00%)	1 / 26 (3.85%)	
occurrences causally related to treatment / all	0 / 0	0 / 1	
deaths causally related to treatment / all	0 / 0	0 / 0	
Obstruction gastric			

subjects affected / exposed	0 / 4 (0.00%)	0 / 26 (0.00%)	
occurrences causally related to treatment / all	0 / 0	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Pancreatitis			
subjects affected / exposed	0 / 4 (0.00%)	0 / 26 (0.00%)	
occurrences causally related to treatment / all	0 / 0	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Vomiting			
subjects affected / exposed	0 / 4 (0.00%)	2 / 26 (7.69%)	
occurrences causally related to treatment / all	0 / 0	0 / 2	
deaths causally related to treatment / all	0 / 0	0 / 0	
Hepatobiliary disorders			
Biliary obstruction			
subjects affected / exposed	0 / 4 (0.00%)	0 / 26 (0.00%)	
occurrences causally related to treatment / all	0 / 0	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Bile duct stenosis			
subjects affected / exposed	0 / 4 (0.00%)	0 / 26 (0.00%)	
occurrences causally related to treatment / all	0 / 0	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Cholangitis			
subjects affected / exposed	1 / 4 (25.00%)	0 / 26 (0.00%)	
occurrences causally related to treatment / all	0 / 1	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Skin and subcutaneous tissue disorders			
Cutaneous calcification			
subjects affected / exposed	0 / 4 (0.00%)	0 / 26 (0.00%)	
occurrences causally related to treatment / all	0 / 0	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Stevens-Johnson syndrome			
subjects affected / exposed	0 / 4 (0.00%)	0 / 26 (0.00%)	
occurrences causally related to treatment / all	0 / 0	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Renal and urinary disorders			

Acute kidney injury			
subjects affected / exposed	0 / 4 (0.00%)	0 / 26 (0.00%)	
occurrences causally related to treatment / all	0 / 0	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Hydronephrosis			
subjects affected / exposed	0 / 4 (0.00%)	0 / 26 (0.00%)	
occurrences causally related to treatment / all	0 / 0	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Ureteric obstruction			
subjects affected / exposed	0 / 4 (0.00%)	1 / 26 (3.85%)	
occurrences causally related to treatment / all	0 / 0	0 / 1	
deaths causally related to treatment / all	0 / 0	0 / 0	
Urinary tract obstruction			
subjects affected / exposed	0 / 4 (0.00%)	0 / 26 (0.00%)	
occurrences causally related to treatment / all	0 / 0	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Musculoskeletal and connective tissue disorders			
Back pain			
subjects affected / exposed	0 / 4 (0.00%)	1 / 26 (3.85%)	
occurrences causally related to treatment / all	0 / 0	0 / 1	
deaths causally related to treatment / all	0 / 0	0 / 0	
Pathological fracture			
subjects affected / exposed	0 / 4 (0.00%)	0 / 26 (0.00%)	
occurrences causally related to treatment / all	0 / 0	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Rhabdomyolysis			
subjects affected / exposed	0 / 4 (0.00%)	1 / 26 (3.85%)	
occurrences causally related to treatment / all	0 / 0	1 / 1	
deaths causally related to treatment / all	0 / 0	0 / 0	
Infections and infestations			
Cellulitis			
subjects affected / exposed	0 / 4 (0.00%)	1 / 26 (3.85%)	
occurrences causally related to treatment / all	0 / 0	0 / 1	
deaths causally related to treatment / all	0 / 0	0 / 0	

Fascial infection			
subjects affected / exposed	0 / 4 (0.00%)	1 / 26 (3.85%)	
occurrences causally related to treatment / all	0 / 0	1 / 1	
deaths causally related to treatment / all	0 / 0	0 / 0	
Implant site infection			
subjects affected / exposed	0 / 4 (0.00%)	0 / 26 (0.00%)	
occurrences causally related to treatment / all	0 / 0	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Pneumonia			
subjects affected / exposed	0 / 4 (0.00%)	0 / 26 (0.00%)	
occurrences causally related to treatment / all	0 / 0	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Sepsis			
subjects affected / exposed	0 / 4 (0.00%)	0 / 26 (0.00%)	
occurrences causally related to treatment / all	0 / 0	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Pyelonephritis acute			
subjects affected / exposed	0 / 4 (0.00%)	0 / 26 (0.00%)	
occurrences causally related to treatment / all	0 / 0	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Urinary tract infection			
subjects affected / exposed	0 / 4 (0.00%)	0 / 26 (0.00%)	
occurrences causally related to treatment / all	0 / 0	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Metabolism and nutrition disorders			
Decreased appetite			
subjects affected / exposed	0 / 4 (0.00%)	1 / 26 (3.85%)	
occurrences causally related to treatment / all	0 / 0	0 / 1	
deaths causally related to treatment / all	0 / 0	0 / 0	
Dehydration			
subjects affected / exposed	1 / 4 (25.00%)	0 / 26 (0.00%)	
occurrences causally related to treatment / all	0 / 1	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Hypercalcaemia			

subjects affected / exposed	0 / 4 (0.00%)	2 / 26 (7.69%)	
occurrences causally related to treatment / all	0 / 0	0 / 2	
deaths causally related to treatment / all	0 / 0	0 / 0	
Hyponatraemia			
subjects affected / exposed	0 / 4 (0.00%)	0 / 26 (0.00%)	
occurrences causally related to treatment / all	0 / 0	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	

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

Non-serious adverse events	Cohort A: FGFR1-3 in-frame fusions or FGFR2 arrangements	Cohort B: known or likely activating FGFR1-3 mutations	Total
Total subjects affected by non-serious adverse events			
subjects affected / exposed	49 / 49 (100.00%)	32 / 32 (100.00%)	111 / 111 (100.00%)
Vascular disorders			
Hypertension			
subjects affected / exposed	3 / 49 (6.12%)	0 / 32 (0.00%)	4 / 111 (3.60%)
occurrences (all)	3	0	4
Hypotension			
subjects affected / exposed	2 / 49 (4.08%)	4 / 32 (12.50%)	6 / 111 (5.41%)
occurrences (all)	2	6	8
General disorders and administration site conditions			
Asthenia			
subjects affected / exposed	8 / 49 (16.33%)	11 / 32 (34.38%)	27 / 111 (24.32%)
occurrences (all)	8	15	32
Fatigue			
subjects affected / exposed	10 / 49 (20.41%)	9 / 32 (28.13%)	23 / 111 (20.72%)
occurrences (all)	14	11	29
Malaise			
subjects affected / exposed	0 / 49 (0.00%)	4 / 32 (12.50%)	5 / 111 (4.50%)
occurrences (all)	0	4	5
Oedema peripheral			
subjects affected / exposed	6 / 49 (12.24%)	5 / 32 (15.63%)	13 / 111 (11.71%)
occurrences (all)	6	5	13
Pyrexia			

subjects affected / exposed occurrences (all)	2 / 49 (4.08%) 2	3 / 32 (9.38%) 3	8 / 111 (7.21%) 8
Xerosis subjects affected / exposed occurrences (all)	0 / 49 (0.00%) 0	2 / 32 (6.25%) 2	3 / 111 (2.70%) 3
Respiratory, thoracic and mediastinal disorders			
Cough subjects affected / exposed occurrences (all)	3 / 49 (6.12%) 3	6 / 32 (18.75%) 7	11 / 111 (9.91%) 12
Dyspnoea subjects affected / exposed occurrences (all)	3 / 49 (6.12%) 3	4 / 32 (12.50%) 5	9 / 111 (8.11%) 10
Epistaxis subjects affected / exposed occurrences (all)	5 / 49 (10.20%) 5	3 / 32 (9.38%) 3	9 / 111 (8.11%) 9
Nasal dryness subjects affected / exposed occurrences (all)	1 / 49 (2.04%) 1	2 / 32 (6.25%) 2	3 / 111 (2.70%) 3
Oropharyngeal pain subjects affected / exposed occurrences (all)	3 / 49 (6.12%) 3	1 / 32 (3.13%) 1	4 / 111 (3.60%) 4
Psychiatric disorders			
Agitation subjects affected / exposed occurrences (all)	3 / 49 (6.12%) 3	0 / 32 (0.00%) 0	3 / 111 (2.70%) 3
Depression subjects affected / exposed occurrences (all)	3 / 49 (6.12%) 3	0 / 32 (0.00%) 0	3 / 111 (2.70%) 3
Insomnia subjects affected / exposed occurrences (all)	4 / 49 (8.16%) 4	0 / 32 (0.00%) 0	4 / 111 (3.60%) 4
Investigations			
Alanine aminotransferase increased subjects affected / exposed occurrences (all)	6 / 49 (12.24%) 8	5 / 32 (15.63%) 5	14 / 111 (12.61%) 16
Blood alkaline phosphatase increased			

subjects affected / exposed	2 / 49 (4.08%)	0 / 32 (0.00%)	4 / 111 (3.60%)
occurrences (all)	2	0	4
Blood creatinine increased			
subjects affected / exposed	10 / 49 (20.41%)	3 / 32 (9.38%)	18 / 111 (16.22%)
occurrences (all)	10	3	18
Aspartate aminotransferase increased			
subjects affected / exposed	5 / 49 (10.20%)	4 / 32 (12.50%)	14 / 111 (12.61%)
occurrences (all)	9	5	20
Hepatic enzyme increased			
subjects affected / exposed	0 / 49 (0.00%)	0 / 32 (0.00%)	1 / 111 (0.90%)
occurrences (all)	0	0	1
Lipase increased			
subjects affected / exposed	2 / 49 (4.08%)	1 / 32 (3.13%)	5 / 111 (4.50%)
occurrences (all)	3	1	7
Neutrophil count decreased			
subjects affected / exposed	1 / 49 (2.04%)	2 / 32 (6.25%)	3 / 111 (2.70%)
occurrences (all)	1	2	3
Weight decreased			
subjects affected / exposed	8 / 49 (16.33%)	6 / 32 (18.75%)	16 / 111 (14.41%)
occurrences (all)	9	6	17
Injury, poisoning and procedural complications			
Fall			
subjects affected / exposed	3 / 49 (6.12%)	1 / 32 (3.13%)	4 / 111 (3.60%)
occurrences (all)	4	1	5
Nervous system disorders			
Dysgeusia			
subjects affected / exposed	10 / 49 (20.41%)	14 / 32 (43.75%)	30 / 111 (27.03%)
occurrences (all)	12	17	36
Dizziness			
subjects affected / exposed	5 / 49 (10.20%)	1 / 32 (3.13%)	7 / 111 (6.31%)
occurrences (all)	5	3	9
Headache			
subjects affected / exposed	5 / 49 (10.20%)	0 / 32 (0.00%)	7 / 111 (6.31%)
occurrences (all)	5	0	7
Presyncope			

subjects affected / exposed occurrences (all)	1 / 49 (2.04%) 1	2 / 32 (6.25%) 2	3 / 111 (2.70%) 3
Taste disorder subjects affected / exposed occurrences (all)	1 / 49 (2.04%) 1	2 / 32 (6.25%) 2	5 / 111 (4.50%) 5
Blood and lymphatic system disorders			
Anaemia subjects affected / exposed occurrences (all)	8 / 49 (16.33%) 9	4 / 32 (12.50%) 4	14 / 111 (12.61%) 15
Thrombocytopenia subjects affected / exposed occurrences (all)	2 / 49 (4.08%) 3	2 / 32 (6.25%) 2	4 / 111 (3.60%) 5
Eye disorders			
Dry eye subjects affected / exposed occurrences (all)	12 / 49 (24.49%) 13	9 / 32 (28.13%) 10	25 / 111 (22.52%) 28
Keratitis subjects affected / exposed occurrences (all)	2 / 49 (4.08%) 2	0 / 32 (0.00%) 0	4 / 111 (3.60%) 4
Serous retinal detachment subjects affected / exposed occurrences (all)	5 / 49 (10.20%) 6	3 / 32 (9.38%) 3	9 / 111 (8.11%) 10
Vision blurred subjects affected / exposed occurrences (all)	5 / 49 (10.20%) 5	3 / 32 (9.38%) 4	8 / 111 (7.21%) 9
Gastrointestinal disorders			
Abdominal pain upper subjects affected / exposed occurrences (all)	3 / 49 (6.12%) 3	1 / 32 (3.13%) 2	7 / 111 (6.31%) 8
Abdominal pain subjects affected / exposed occurrences (all)	7 / 49 (14.29%) 10	3 / 32 (9.38%) 3	15 / 111 (13.51%) 18
Cheilitis subjects affected / exposed occurrences (all)	1 / 49 (2.04%) 1	2 / 32 (6.25%) 2	3 / 111 (2.70%) 3
Constipation			

subjects affected / exposed	18 / 49 (36.73%)	13 / 32 (40.63%)	37 / 111 (33.33%)
occurrences (all)	20	18	44
Dyspepsia			
subjects affected / exposed	1 / 49 (2.04%)	4 / 32 (12.50%)	6 / 111 (5.41%)
occurrences (all)	1	5	7
Dry mouth			
subjects affected / exposed	13 / 49 (26.53%)	11 / 32 (34.38%)	32 / 111 (28.83%)
occurrences (all)	15	13	37
Diarrhoea			
subjects affected / exposed	19 / 49 (38.78%)	17 / 32 (53.13%)	43 / 111 (38.74%)
occurrences (all)	28	30	65
Dysphagia			
subjects affected / exposed	3 / 49 (6.12%)	0 / 32 (0.00%)	3 / 111 (2.70%)
occurrences (all)	3	0	3
Nausea			
subjects affected / exposed	11 / 49 (22.45%)	8 / 32 (25.00%)	27 / 111 (24.32%)
occurrences (all)	12	12	33
Stomatitis			
subjects affected / exposed	27 / 49 (55.10%)	17 / 32 (53.13%)	59 / 111 (53.15%)
occurrences (all)	39	25	87
Vomiting			
subjects affected / exposed	10 / 49 (20.41%)	5 / 32 (15.63%)	20 / 111 (18.02%)
occurrences (all)	14	6	25
Hepatobiliary disorders			
Hepatic function abnormal			
subjects affected / exposed	0 / 49 (0.00%)	0 / 32 (0.00%)	2 / 111 (1.80%)
occurrences (all)	0	0	3
Skin and subcutaneous tissue disorders			
Alopecia			
subjects affected / exposed	20 / 49 (40.82%)	17 / 32 (53.13%)	45 / 111 (40.54%)
occurrences (all)	20	21	49
Dry skin			
subjects affected / exposed	6 / 49 (12.24%)	6 / 32 (18.75%)	18 / 111 (16.22%)
occurrences (all)	7	7	21
Erythema			

subjects affected / exposed	4 / 49 (8.16%)	0 / 32 (0.00%)	4 / 111 (3.60%)
occurrences (all)	5	0	5
Nail discolouration			
subjects affected / exposed	7 / 49 (14.29%)	4 / 32 (12.50%)	13 / 111 (11.71%)
occurrences (all)	7	4	13
Nail disorder			
subjects affected / exposed	4 / 49 (8.16%)	4 / 32 (12.50%)	10 / 111 (9.01%)
occurrences (all)	4	4	10
Onychalgia			
subjects affected / exposed	2 / 49 (4.08%)	2 / 32 (6.25%)	4 / 111 (3.60%)
occurrences (all)	2	2	4
Onycholysis			
subjects affected / exposed	2 / 49 (4.08%)	5 / 32 (15.63%)	10 / 111 (9.01%)
occurrences (all)	3	6	12
Onychomadesis			
subjects affected / exposed	7 / 49 (14.29%)	6 / 32 (18.75%)	17 / 111 (15.32%)
occurrences (all)	8	6	18
Palmar-plantar erythrodysaesthesia syndrome			
subjects affected / exposed	13 / 49 (26.53%)	8 / 32 (25.00%)	26 / 111 (23.42%)
occurrences (all)	15	11	31
Pruritus			
subjects affected / exposed	2 / 49 (4.08%)	4 / 32 (12.50%)	6 / 111 (5.41%)
occurrences (all)	2	4	6
Rash			
subjects affected / exposed	3 / 49 (6.12%)	2 / 32 (6.25%)	5 / 111 (4.50%)
occurrences (all)	3	2	5
Skin exfoliation			
subjects affected / exposed	0 / 49 (0.00%)	2 / 32 (6.25%)	2 / 111 (1.80%)
occurrences (all)	0	2	2
Renal and urinary disorders			
Chronic kidney disease			
subjects affected / exposed	0 / 49 (0.00%)	2 / 32 (6.25%)	2 / 111 (1.80%)
occurrences (all)	0	5	5
Urinary retention			

subjects affected / exposed occurrences (all)	4 / 49 (8.16%) 4	2 / 32 (6.25%) 2	6 / 111 (5.41%) 6
Renal failure subjects affected / exposed occurrences (all)	1 / 49 (2.04%) 1	3 / 32 (9.38%) 3	5 / 111 (4.50%) 5
Musculoskeletal and connective tissue disorders			
Back pain subjects affected / exposed occurrences (all)	3 / 49 (6.12%) 3	4 / 32 (12.50%) 4	8 / 111 (7.21%) 8
Arthritis subjects affected / exposed occurrences (all)	0 / 49 (0.00%) 0	2 / 32 (6.25%) 2	2 / 111 (1.80%) 2
Arthralgia subjects affected / exposed occurrences (all)	11 / 49 (22.45%) 12	11 / 32 (34.38%) 14	23 / 111 (20.72%) 27
Muscle spasms subjects affected / exposed occurrences (all)	3 / 49 (6.12%) 3	1 / 32 (3.13%) 2	4 / 111 (3.60%) 5
Musculoskeletal chest pain subjects affected / exposed occurrences (all)	0 / 49 (0.00%) 0	2 / 32 (6.25%) 2	2 / 111 (1.80%) 2
Myalgia subjects affected / exposed occurrences (all)	4 / 49 (8.16%) 4	4 / 32 (12.50%) 4	10 / 111 (9.01%) 10
Pain in extremity subjects affected / exposed occurrences (all)	7 / 49 (14.29%) 8	0 / 32 (0.00%) 0	10 / 111 (9.01%) 11
Osteoarthritis subjects affected / exposed occurrences (all)	1 / 49 (2.04%) 1	2 / 32 (6.25%) 2	3 / 111 (2.70%) 3
Infections and infestations			
COVID-19 subjects affected / exposed occurrences (all)	4 / 49 (8.16%) 4	0 / 32 (0.00%) 0	4 / 111 (3.60%) 4
Conjunctivitis			

subjects affected / exposed	2 / 49 (4.08%)	2 / 32 (6.25%)	7 / 111 (6.31%)
occurrences (all)	2	2	8
Cystitis			
subjects affected / exposed	3 / 49 (6.12%)	1 / 32 (3.13%)	4 / 111 (3.60%)
occurrences (all)	3	1	4
Nail infection			
subjects affected / exposed	0 / 49 (0.00%)	2 / 32 (6.25%)	3 / 111 (2.70%)
occurrences (all)	0	2	3
Paronychia			
subjects affected / exposed	5 / 49 (10.20%)	6 / 32 (18.75%)	14 / 111 (12.61%)
occurrences (all)	5	7	15
Urinary tract infection			
subjects affected / exposed	6 / 49 (12.24%)	5 / 32 (15.63%)	15 / 111 (13.51%)
occurrences (all)	6	5	16
Metabolism and nutrition disorders			
Decreased appetite			
subjects affected / exposed	10 / 49 (20.41%)	12 / 32 (37.50%)	27 / 111 (24.32%)
occurrences (all)	14	15	34
Dehydration			
subjects affected / exposed	3 / 49 (6.12%)	1 / 32 (3.13%)	4 / 111 (3.60%)
occurrences (all)	3	1	4
Hyperglycaemia			
subjects affected / exposed	3 / 49 (6.12%)	1 / 32 (3.13%)	4 / 111 (3.60%)
occurrences (all)	3	1	4
Hypercalcaemia			
subjects affected / exposed	2 / 49 (4.08%)	3 / 32 (9.38%)	8 / 111 (7.21%)
occurrences (all)	4	3	15
Hyperphosphataemia			
subjects affected / exposed	45 / 49 (91.84%)	27 / 32 (84.38%)	93 / 111 (83.78%)
occurrences (all)	72	51	154
Hypokalaemia			
subjects affected / exposed	3 / 49 (6.12%)	1 / 32 (3.13%)	4 / 111 (3.60%)
occurrences (all)	3	1	4
Hypomagnesaemia			
subjects affected / exposed	5 / 49 (10.20%)	1 / 32 (3.13%)	7 / 111 (6.31%)
occurrences (all)	5	2	8

Hyponatraemia subjects affected / exposed occurrences (all)	4 / 49 (8.16%) 5	1 / 32 (3.13%) 1	6 / 111 (5.41%) 7
Hypophosphataemia subjects affected / exposed occurrences (all)	4 / 49 (8.16%) 4	3 / 32 (9.38%) 3	10 / 111 (9.01%) 12
Vitamin D deficiency subjects affected / exposed occurrences (all)	1 / 49 (2.04%) 2	1 / 32 (3.13%) 1	4 / 111 (3.60%) 5

Non-serious adverse events	Other	Cohort C: other FGFR mutations or arrangements	
Total subjects affected by non-serious adverse events subjects affected / exposed	4 / 4 (100.00%)	26 / 26 (100.00%)	
Vascular disorders			
Hypertension subjects affected / exposed occurrences (all)	0 / 4 (0.00%) 0	1 / 26 (3.85%) 1	
Hypotension subjects affected / exposed occurrences (all)	0 / 4 (0.00%) 0	0 / 26 (0.00%) 0	
General disorders and administration site conditions			
Asthenia subjects affected / exposed occurrences (all)	0 / 4 (0.00%) 0	8 / 26 (30.77%) 9	
Fatigue subjects affected / exposed occurrences (all)	0 / 4 (0.00%) 0	4 / 26 (15.38%) 4	
Malaise subjects affected / exposed occurrences (all)	1 / 4 (25.00%) 1	0 / 26 (0.00%) 0	
Oedema peripheral subjects affected / exposed occurrences (all)	0 / 4 (0.00%) 0	2 / 26 (7.69%) 2	
Pyrexia subjects affected / exposed occurrences (all)	2 / 4 (50.00%) 2	1 / 26 (3.85%) 1	

Xerosis subjects affected / exposed occurrences (all)	0 / 4 (0.00%) 0	1 / 26 (3.85%) 1	
Respiratory, thoracic and mediastinal disorders Cough subjects affected / exposed occurrences (all) Dyspnoea subjects affected / exposed occurrences (all) Epistaxis subjects affected / exposed occurrences (all) Nasal dryness subjects affected / exposed occurrences (all) Oropharyngeal pain subjects affected / exposed occurrences (all)	0 / 4 (0.00%) 0 0 / 4 (0.00%) 0 0 / 4 (0.00%) 0 0 / 4 (0.00%) 0 0 / 4 (0.00%) 0	2 / 26 (7.69%) 2 2 / 26 (7.69%) 2 1 / 26 (3.85%) 1 0 / 26 (0.00%) 0 0 / 26 (0.00%) 0	
Psychiatric disorders Agitation subjects affected / exposed occurrences (all) Depression subjects affected / exposed occurrences (all) Insomnia subjects affected / exposed occurrences (all)	0 / 4 (0.00%) 0 0 / 4 (0.00%) 0 0 / 4 (0.00%) 0	0 / 26 (0.00%) 0 0 / 26 (0.00%) 0 0 / 26 (0.00%) 0	
Investigations Alanine aminotransferase increased subjects affected / exposed occurrences (all) Blood alkaline phosphatase increased subjects affected / exposed occurrences (all)	0 / 4 (0.00%) 0 0 / 4 (0.00%) 0	3 / 26 (11.54%) 3 2 / 26 (7.69%) 2	

Blood creatinine increased subjects affected / exposed occurrences (all)	0 / 4 (0.00%) 0	5 / 26 (19.23%) 5	
Aspartate aminotransferase increased subjects affected / exposed occurrences (all)	0 / 4 (0.00%) 0	5 / 26 (19.23%) 6	
Hepatic enzyme increased subjects affected / exposed occurrences (all)	1 / 4 (25.00%) 1	0 / 26 (0.00%) 0	
Lipase increased subjects affected / exposed occurrences (all)	0 / 4 (0.00%) 0	2 / 26 (7.69%) 3	
Neutrophil count decreased subjects affected / exposed occurrences (all)	0 / 4 (0.00%) 0	0 / 26 (0.00%) 0	
Weight decreased subjects affected / exposed occurrences (all)	0 / 4 (0.00%) 0	2 / 26 (7.69%) 2	
Injury, poisoning and procedural complications Fall subjects affected / exposed occurrences (all)	0 / 4 (0.00%) 0	0 / 26 (0.00%) 0	
Nervous system disorders Dysgeusia subjects affected / exposed occurrences (all)	0 / 4 (0.00%) 0	6 / 26 (23.08%) 7	
Dizziness subjects affected / exposed occurrences (all)	0 / 4 (0.00%) 0	1 / 26 (3.85%) 1	
Headache subjects affected / exposed occurrences (all)	0 / 4 (0.00%) 0	2 / 26 (7.69%) 2	
Presyncope subjects affected / exposed occurrences (all)	0 / 4 (0.00%) 0	0 / 26 (0.00%) 0	
Taste disorder			

subjects affected / exposed occurrences (all)	0 / 4 (0.00%) 0	2 / 26 (7.69%) 2	
Blood and lymphatic system disorders			
Anaemia			
subjects affected / exposed	0 / 4 (0.00%)	2 / 26 (7.69%)	
occurrences (all)	0	2	
Thrombocytopenia			
subjects affected / exposed	0 / 4 (0.00%)	0 / 26 (0.00%)	
occurrences (all)	0	0	
Eye disorders			
Dry eye			
subjects affected / exposed	0 / 4 (0.00%)	4 / 26 (15.38%)	
occurrences (all)	0	5	
Keratitis			
subjects affected / exposed	0 / 4 (0.00%)	2 / 26 (7.69%)	
occurrences (all)	0	2	
Serous retinal detachment			
subjects affected / exposed	0 / 4 (0.00%)	1 / 26 (3.85%)	
occurrences (all)	0	1	
Vision blurred			
subjects affected / exposed	0 / 4 (0.00%)	0 / 26 (0.00%)	
occurrences (all)	0	0	
Gastrointestinal disorders			
Abdominal pain upper			
subjects affected / exposed	0 / 4 (0.00%)	3 / 26 (11.54%)	
occurrences (all)	0	3	
Abdominal pain			
subjects affected / exposed	0 / 4 (0.00%)	5 / 26 (19.23%)	
occurrences (all)	0	5	
Cheilitis			
subjects affected / exposed	0 / 4 (0.00%)	0 / 26 (0.00%)	
occurrences (all)	0	0	
Constipation			
subjects affected / exposed	0 / 4 (0.00%)	6 / 26 (23.08%)	
occurrences (all)	0	6	
Dyspepsia			

subjects affected / exposed	0 / 4 (0.00%)	1 / 26 (3.85%)	
occurrences (all)	0	1	
Dry mouth			
subjects affected / exposed	1 / 4 (25.00%)	7 / 26 (26.92%)	
occurrences (all)	1	8	
Diarrhoea			
subjects affected / exposed	0 / 4 (0.00%)	7 / 26 (26.92%)	
occurrences (all)	0	7	
Dysphagia			
subjects affected / exposed	0 / 4 (0.00%)	0 / 26 (0.00%)	
occurrences (all)	0	0	
Nausea			
subjects affected / exposed	2 / 4 (50.00%)	6 / 26 (23.08%)	
occurrences (all)	2	7	
Stomatitis			
subjects affected / exposed	2 / 4 (50.00%)	13 / 26 (50.00%)	
occurrences (all)	2	21	
Vomiting			
subjects affected / exposed	0 / 4 (0.00%)	5 / 26 (19.23%)	
occurrences (all)	0	5	
Hepatobiliary disorders			
Hepatic function abnormal			
subjects affected / exposed	1 / 4 (25.00%)	1 / 26 (3.85%)	
occurrences (all)	2	1	
Skin and subcutaneous tissue disorders			
Alopecia			
subjects affected / exposed	0 / 4 (0.00%)	8 / 26 (30.77%)	
occurrences (all)	0	8	
Dry skin			
subjects affected / exposed	1 / 4 (25.00%)	5 / 26 (19.23%)	
occurrences (all)	1	6	
Erythema			
subjects affected / exposed	0 / 4 (0.00%)	0 / 26 (0.00%)	
occurrences (all)	0	0	
Nail discolouration			

subjects affected / exposed	0 / 4 (0.00%)	2 / 26 (7.69%)	
occurrences (all)	0	2	
Nail disorder			
subjects affected / exposed	0 / 4 (0.00%)	2 / 26 (7.69%)	
occurrences (all)	0	2	
Onychalgia			
subjects affected / exposed	0 / 4 (0.00%)	0 / 26 (0.00%)	
occurrences (all)	0	0	
Onycholysis			
subjects affected / exposed	0 / 4 (0.00%)	3 / 26 (11.54%)	
occurrences (all)	0	3	
Onychomadesis			
subjects affected / exposed	0 / 4 (0.00%)	4 / 26 (15.38%)	
occurrences (all)	0	4	
Palmar-plantar erythrodysesthesia syndrome			
subjects affected / exposed	0 / 4 (0.00%)	5 / 26 (19.23%)	
occurrences (all)	0	5	
Pruritus			
subjects affected / exposed	0 / 4 (0.00%)	0 / 26 (0.00%)	
occurrences (all)	0	0	
Rash			
subjects affected / exposed	0 / 4 (0.00%)	0 / 26 (0.00%)	
occurrences (all)	0	0	
Skin exfoliation			
subjects affected / exposed	0 / 4 (0.00%)	0 / 26 (0.00%)	
occurrences (all)	0	0	
Renal and urinary disorders			
Chronic kidney disease			
subjects affected / exposed	0 / 4 (0.00%)	0 / 26 (0.00%)	
occurrences (all)	0	0	
Urinary retention			
subjects affected / exposed	0 / 4 (0.00%)	0 / 26 (0.00%)	
occurrences (all)	0	0	
Renal failure			

subjects affected / exposed occurrences (all)	0 / 4 (0.00%) 0	1 / 26 (3.85%) 1	
Musculoskeletal and connective tissue disorders			
Back pain			
subjects affected / exposed	0 / 4 (0.00%)	1 / 26 (3.85%)	
occurrences (all)	0	1	
Arthritis			
subjects affected / exposed	0 / 4 (0.00%)	0 / 26 (0.00%)	
occurrences (all)	0	0	
Arthralgia			
subjects affected / exposed	0 / 4 (0.00%)	1 / 26 (3.85%)	
occurrences (all)	0	1	
Muscle spasms			
subjects affected / exposed	0 / 4 (0.00%)	0 / 26 (0.00%)	
occurrences (all)	0	0	
Musculoskeletal chest pain			
subjects affected / exposed	0 / 4 (0.00%)	0 / 26 (0.00%)	
occurrences (all)	0	0	
Myalgia			
subjects affected / exposed	0 / 4 (0.00%)	2 / 26 (7.69%)	
occurrences (all)	0	2	
Pain in extremity			
subjects affected / exposed	0 / 4 (0.00%)	3 / 26 (11.54%)	
occurrences (all)	0	3	
Osteoarthritis			
subjects affected / exposed	0 / 4 (0.00%)	0 / 26 (0.00%)	
occurrences (all)	0	0	
Infections and infestations			
COVID-19			
subjects affected / exposed	0 / 4 (0.00%)	0 / 26 (0.00%)	
occurrences (all)	0	0	
Conjunctivitis			
subjects affected / exposed	0 / 4 (0.00%)	3 / 26 (11.54%)	
occurrences (all)	0	4	
Cystitis			

subjects affected / exposed	0 / 4 (0.00%)	0 / 26 (0.00%)	
occurrences (all)	0	0	
Nail infection			
subjects affected / exposed	0 / 4 (0.00%)	1 / 26 (3.85%)	
occurrences (all)	0	1	
Paronychia			
subjects affected / exposed	0 / 4 (0.00%)	3 / 26 (11.54%)	
occurrences (all)	0	3	
Urinary tract infection			
subjects affected / exposed	0 / 4 (0.00%)	4 / 26 (15.38%)	
occurrences (all)	0	5	
Metabolism and nutrition disorders			
Decreased appetite			
subjects affected / exposed	0 / 4 (0.00%)	5 / 26 (19.23%)	
occurrences (all)	0	5	
Dehydration			
subjects affected / exposed	0 / 4 (0.00%)	0 / 26 (0.00%)	
occurrences (all)	0	0	
Hyperglycaemia			
subjects affected / exposed	0 / 4 (0.00%)	0 / 26 (0.00%)	
occurrences (all)	0	0	
Hypercalcaemia			
subjects affected / exposed	0 / 4 (0.00%)	3 / 26 (11.54%)	
occurrences (all)	0	8	
Hyperphosphataemia			
subjects affected / exposed	4 / 4 (100.00%)	17 / 26 (65.38%)	
occurrences (all)	4	27	
Hypokalaemia			
subjects affected / exposed	0 / 4 (0.00%)	0 / 26 (0.00%)	
occurrences (all)	0	0	
Hypomagnesaemia			
subjects affected / exposed	1 / 4 (25.00%)	0 / 26 (0.00%)	
occurrences (all)	1	0	
Hyponatraemia			
subjects affected / exposed	1 / 4 (25.00%)	0 / 26 (0.00%)	
occurrences (all)	1	0	

Hypophosphataemia			
subjects affected / exposed	1 / 4 (25.00%)	2 / 26 (7.69%)	
occurrences (all)	1	4	
Vitamin D deficiency			
subjects affected / exposed	0 / 4 (0.00%)	2 / 26 (7.69%)	
occurrences (all)	0	2	

More information

Substantial protocol amendments (globally)

Were there any global substantial amendments to the protocol? Yes

Date	Amendment
14 February 2019	The protocol was amended to incorporate updates based on Voluntary Harmonization Procedure review of other pemigatinib protocols and to update safety information based on the revised/updated Investigator's Brochure.
14 January 2020	The protocol was amended to incorporate updates of the cohort definitions and other changes based on Food and Drug Administration review of other pemigatinib protocols.
15 February 2021	The protocol was amended to incorporate updates regarding tumor biopsy timing and other clarifications.

Notes:

Interruptions (globally)

Were there any global interruptions to the trial? No

Limitations and caveats

None reported