



## Clinical trial results:

### A Phase 2 Study of INCB086550 (Oral PD-L1 Inhibitor) in Participants Who Are Immune Checkpoint Inhibitor–Naïve With Selected Solid Tumors

#### Summary

EudraCT number	2020-000157-27
Trial protocol	HU BG RO
Global end of trial date	26 March 2024

#### Results information

Result version number	v1 (current)
This version publication date	15 March 2025
First version publication date	15 March 2025

#### Trial information

##### Trial identification

Sponsor protocol code	INCB 86550-203
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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 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	26 March 2024
Is this the analysis of the primary completion data?	No

Global end of trial reached?	Yes
Global end of trial date	26 March 2024
Was the trial ended prematurely?	Yes

Notes:

## General information about the trial

Main objective of the trial:

This study was conducted to evaluate the efficacy and safety of INCB086550, a first-in-class oral inhibitor of programmed death-ligand 1 (PD-L1), as an initial immune checkpoint inhibitor therapy in participants with select solid tumors.

Protection of trial subjects:

This study was 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 conducted.

Background therapy: -

Evidence for comparator: -

Actual start date of recruitment	02 September 2021
Long term follow-up planned	Yes
Long term follow-up rationale	Efficacy, Safety
Long term follow-up duration	6 Months
Independent data monitoring committee (IDMC) involvement?	Yes

Notes:

## Population of trial subjects

### Subjects enrolled per country

Country: Number of subjects enrolled	Hungary: 1
Country: Number of subjects enrolled	Korea, Republic of: 2
Country: Number of subjects enrolled	Ukraine: 13
Worldwide total number of subjects	16
EEA total number of subjects	1

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)	10
From 65 to 84 years	6
85 years and over	0

## Subject disposition

### Recruitment

Recruitment details: -

### Pre-assignment

Screening details:

This study was conducted at 10 study centers in Hungary, Korea, and Ukraine.

### 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>	NSCLC 400 mg BID

Arm description:

Participants with non-small cell lung cancer (NSCLC) received INCB086550 orally in 28-day cycles at a dose of 400 milligrams (mg) twice daily (BID) for up to 2 years as long as they received benefit and didn't meet any criteria for study withdrawal. Participants who achieved a complete response could have continued to receive INCB086550 for an additional 4 cycles (with a minimum of 1 year of treatment) upon medical monitor consultation.

Arm type	Experimental
Investigational medicinal product name	INCB086550
Investigational medicinal product code	
Other name	
Pharmaceutical forms	Tablet
Routes of administration	Oral use

Dosage and administration details:

administered orally in 100 mg tablets

<b>Arm title</b>	UC 400 mg BID
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Arm description:

Participants with urothelial carcinoma (UC) received INCB086550 orally in 28-day cycles at a dose of 400 mg BID for up to 2 years as long as they received benefit and didn't meet any criteria for study withdrawal. Participants who achieved a complete response could have continued to receive INCB086550 for an additional 4 cycles (with a minimum of 1 year of treatment) upon medical monitor consultation.

Arm type	Experimental
Investigational medicinal product name	INCB086550
Investigational medicinal product code	
Other name	
Pharmaceutical forms	Tablet
Routes of administration	Oral use

Dosage and administration details:

administered orally in 100 mg tablets

<b>Arm title</b>	RCC 400 mg BID
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Arm description:

Participants with renal cell carcinoma (RC) received INCB086550 orally in 28-day cycles at a dose of 400 mg BID for up to 2 years as long as they received benefit and didn't meet any criteria for study withdrawal. Participants who achieved a complete response could have continued to receive INCB086550 for an additional 4 cycles (with a minimum of 1 year of treatment) upon medical monitor consultation.

Arm type	Experimental
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Investigational medicinal product name	INCB086550
Investigational medicinal product code	
Other name	
Pharmaceutical forms	Tablet
Routes of administration	Oral use
Dosage and administration details: administered orally in 100 mg tablets	
<b>Arm title</b>	Melanoma 400 mg BID

**Arm description:**

Participants with melanoma received INCB086550 orally in 28-day cycles at a dose of 400 mg BID for up to 2 years as long as they received benefit and didn't meet any criteria for study withdrawal. Participants who achieved a complete response could have continued to receive INCB086550 for an additional 4 cycles (with a minimum of 1 year of treatment) upon medical monitor consultation.

Arm type	Experimental
Investigational medicinal product name	INCB086550
Investigational medicinal product code	
Other name	
Pharmaceutical forms	Tablet
Routes of administration	Oral use

Dosage and administration details:  
administered orally in 100 mg tablets

<b>Arm title</b>	Melanoma 400 mg BID Intermittent Dose
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**Arm description:**

Participants with melanoma received intermittent INCB086550 orally in 28-day cycles at a dose of 400 mg BID. Participants received INCB086550 for 1 week followed by 1 week off for up to 2 years as long as they received benefit and didn't meet any criteria for study withdrawal. Participants who achieved a complete response could have continued to receive INCB086550 for an additional 4 cycles (with a minimum of 1 year of treatment) upon medical monitor consultation.

Arm type	Experimental
Investigational medicinal product name	INCB086550
Investigational medicinal product code	
Other name	
Pharmaceutical forms	Tablet
Routes of administration	Oral use

Dosage and administration details:  
administered orally in 100 mg tablets

<b>Number of subjects in period 1</b>	NSCLC 400 mg BID	UC 400 mg BID	RCC 400 mg BID
Started	1	1	6
Completed	0	0	1
Not completed	1	1	5
Adverse event, serious fatal	1	-	2
Physician decision	-	1	-
Consent withdrawn by subject	-	-	2
Study Terminated by Sponsor	-	-	-
Lost to follow-up	-	-	-
Disease Progression	-	-	1

Number of subjects in period 1	Melanoma 400 mg BID	Melanoma 400 mg BID Intermittent Dose
Started	7	1
Completed	0	1
Not completed	7	0
Adverse event, serious fatal	2	-
Physician decision	-	-
Consent withdrawn by subject	2	-
Study Terminated by Sponsor	1	-
Lost to follow-up	1	-
Disease Progression	1	-

## Baseline characteristics

### Reporting groups

Reporting group title	NSCLC 400 mg BID
Reporting group description:	
Participants with non-small cell lung cancer (NSCLC) received INCB086550 orally in 28-day cycles at a dose of 400 milligrams (mg) twice daily (BID) for up to 2 years as long as they received benefit and didn't meet any criteria for study withdrawal. Participants who achieved a complete response could have continued to receive INCB086550 for an additional 4 cycles (with a minimum of 1 year of treatment) upon medical monitor consultation.	
Reporting group title	UC 400 mg BID
Reporting group description:	
Participants with urothelial carcinoma (UC) received INCB086550 orally in 28-day cycles at a dose of 400 mg BID for up to 2 years as long as they received benefit and didn't meet any criteria for study withdrawal. Participants who achieved a complete response could have continued to receive INCB086550 for an additional 4 cycles (with a minimum of 1 year of treatment) upon medical monitor consultation.	
Reporting group title	RCC 400 mg BID
Reporting group description:	
Participants with renal cell carcinoma (RC) received INCB086550 orally in 28-day cycles at a dose of 400 mg BID for up to 2 years as long as they received benefit and didn't meet any criteria for study withdrawal. Participants who achieved a complete response could have continued to receive INCB086550 for an additional 4 cycles (with a minimum of 1 year of treatment) upon medical monitor consultation.	
Reporting group title	Melanoma 400 mg BID
Reporting group description:	
Participants with melanoma received INCB086550 orally in 28-day cycles at a dose of 400 mg BID for up to 2 years as long as they received benefit and didn't meet any criteria for study withdrawal. Participants who achieved a complete response could have continued to receive INCB086550 for an additional 4 cycles (with a minimum of 1 year of treatment) upon medical monitor consultation.	
Reporting group title	Melanoma 400 mg BID Intermittent Dose
Reporting group description:	
Participants with melanoma received intermittent INCB086550 orally in 28-day cycles at a dose of 400 mg BID. Participants received INCB086550 for 1 week followed by 1 week off for up to 2 years as long as they received benefit and didn't meet any criteria for study withdrawal. Participants who achieved a complete response could have continued to receive INCB086550 for an additional 4 cycles (with a minimum of 1 year of treatment) upon medical monitor consultation.	

Reporting group values	NSCLC 400 mg BID	UC 400 mg BID	RCC 400 mg BID
Number of subjects	1	1	6
Age Categorical			
Units: participants			
<=18 years	0	0	0
Between 18 and 65 years	0	0	4
>=65 years	1	1	2
Sex: Female, Male			
Units: participants			
Female	0	0	4
Male	1	1	2
Race (NIH/OMB)			
Units: Subjects			
American Indian or Alaska Native	0	0	0
Asian	0	0	2

Native Hawaiian or Other Pacific Islander	0	0	0
Black or African American	0	0	0
White	1	1	4
More than one race	0	0	0
Unknown or Not Reported	0	0	0
Ethnicity (NIH/OMB)			
Units: Subjects			
Hispanic or Latino	0	0	0
Not Hispanic or Latino	1	1	6
Unknown or Not Reported	0	0	0

<b>Reporting group values</b>	Melanoma 400 mg BID	Melanoma 400 mg BID Intermittent Dose	Total
Number of subjects	7	1	16
Age Categorical			
Units: participants			
<=18 years	0	0	0
Between 18 and 65 years	5	1	10
>=65 years	2	0	6
Sex: Female, Male			
Units: participants			
Female	3	1	8
Male	4	0	8
Race (NIH/OMB)			
Units: Subjects			
American Indian or Alaska Native	0	0	0
Asian	0	0	2
Native Hawaiian or Other Pacific Islander	0	0	0
Black or African American	0	0	0
White	7	1	14
More than one race	0	0	0
Unknown or Not Reported	0	0	0
Ethnicity (NIH/OMB)			
Units: Subjects			
Hispanic or Latino	0	0	0
Not Hispanic or Latino	7	1	16
Unknown or Not Reported	0	0	0



## End points

### End points reporting groups

Reporting group title	NSCLC 400 mg BID
Reporting group description:	
Participants with non-small cell lung cancer (NSCLC) received INCB086550 orally in 28-day cycles at a dose of 400 milligrams (mg) twice daily (BID) for up to 2 years as long as they received benefit and didn't meet any criteria for study withdrawal. Participants who achieved a complete response could have continued to receive INCB086550 for an additional 4 cycles (with a minimum of 1 year of treatment) upon medical monitor consultation.	
Reporting group title	UC 400 mg BID
Reporting group description:	
Participants with urothelial carcinoma (UC) received INCB086550 orally in 28-day cycles at a dose of 400 mg BID for up to 2 years as long as they received benefit and didn't meet any criteria for study withdrawal. Participants who achieved a complete response could have continued to receive INCB086550 for an additional 4 cycles (with a minimum of 1 year of treatment) upon medical monitor consultation.	
Reporting group title	RCC 400 mg BID
Reporting group description:	
Participants with renal cell carcinoma (RC) received INCB086550 orally in 28-day cycles at a dose of 400 mg BID for up to 2 years as long as they received benefit and didn't meet any criteria for study withdrawal. Participants who achieved a complete response could have continued to receive INCB086550 for an additional 4 cycles (with a minimum of 1 year of treatment) upon medical monitor consultation.	
Reporting group title	Melanoma 400 mg BID
Reporting group description:	
Participants with melanoma received INCB086550 orally in 28-day cycles at a dose of 400 mg BID for up to 2 years as long as they received benefit and didn't meet any criteria for study withdrawal. Participants who achieved a complete response could have continued to receive INCB086550 for an additional 4 cycles (with a minimum of 1 year of treatment) upon medical monitor consultation.	
Reporting group title	Melanoma 400 mg BID Intermittent Dose
Reporting group description:	
Participants with melanoma received intermittent INCB086550 orally in 28-day cycles at a dose of 400 mg BID. Participants received INCB086550 for 1 week followed by 1 week off for up to 2 years as long as they received benefit and didn't meet any criteria for study withdrawal. Participants who achieved a complete response could have continued to receive INCB086550 for an additional 4 cycles (with a minimum of 1 year of treatment) upon medical monitor consultation.	

### Primary: Objective Response Rate (ORR)

End point title	Objective Response Rate (ORR) <sup>[1]</sup>
End point description:	
ORR was defined as the percentage of participants with a best overall response of complete response (CR) or partial response (PR), confirmed by $\geq 1$ repeat assessment $\geq 28$ days later, according to Response Evaluation Criteria in Solid Tumors version 1.1 (RECIST v1.1), as determined by the investigator. CR: disappearance of all target and non-target lesions and no appearance of any new lesions. Any pathological lymph nodes (whether target or non-target) must have a reduction in the short axis to $< 10$ millimeters (mm). PR: at least a 30% decrease in the sum of the diameters of target lesions, taking as a reference the baseline sum diameters, no new lesions, and no progression of non-target lesions. Confidence intervals were calculated using the Clopper-Pearson method.	
End point type	Primary
End point timeframe:	
up to 733 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	NSCLC 400 mg BID	UC 400 mg BID	RCC 400 mg BID	Melanoma 400 mg BID
Subject group type	Reporting group	Reporting group	Reporting group	Reporting group
Number of subjects analysed	1 <sup>[2]</sup>	1 <sup>[3]</sup>	6 <sup>[4]</sup>	7 <sup>[5]</sup>
Units: percentage of participants				
number (confidence interval 95%)	0.0 (0.0 to 97.5)	100.0 (2.5 to 100.0)	0.0 (0.0 to 45.9)	14.3 (0.4 to 57.9)

Notes:

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

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

[4] - Full Analysis Set: all study participants who received  $\geq 1$  dose of study drug

[5] - Full Analysis Set: all study participants who received  $\geq 1$  dose of study drug

End point values	Melanoma 400 mg BID Intermittent Dose			
Subject group type	Reporting group			
Number of subjects analysed	1 <sup>[6]</sup>			
Units: percentage of participants				
number (confidence interval 95%)	100.0 (2.5 to 100.0)			

Notes:

[6] - Full Analysis Set: all study participants who received  $\geq 1$  dose of study drug

## Statistical analyses

No statistical analyses for this end point

## Secondary: Disease Control Rate (DCR)

End point title	Disease Control Rate (DCR)
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End point description:

DCR was defined as the percentage of participants with a best overall response of CR or PR, confirmed by  $\geq 1$  repeat assessment  $\geq 28$  days later, or stable disease (SD) for  $\geq 12$  weeks, by investigator assessment per RECIST v1.1. CR: disappearance of all target and non-target lesions and no appearance of any new lesions. Any pathological lymph nodes (whether target or non-target) must have a reduction in the short axis to  $< 10$  mm. PR: at least a 30% decrease in the sum of the diameters of target lesions, taking as a reference the baseline sum diameters, no new lesions, and no progression of non-target lesions. Progressive disease (PD): progression of a target or non-target lesion or presence of a new lesion. SD: no change in target lesions to qualify for CR, PR, or PD. Confidence intervals were calculated using the Clopper-Pearson method.

End point type	Secondary
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End point timeframe:

up to 733 days

End point values	NSCLC 400 mg BID	UC 400 mg BID	RCC 400 mg BID	Melanoma 400 mg BID
Subject group type	Reporting group	Reporting group	Reporting group	Reporting group
Number of subjects analysed	1 <sup>[7]</sup>	1 <sup>[8]</sup>	6 <sup>[9]</sup>	7 <sup>[10]</sup>
Units: percentage of participants				
number (confidence interval 95%)	0.0 (0.0 to 97.5)	100.0 (2.5 to 100.0)	50.0 (11.8 to 88.2)	28.6 (3.7 to 71.0)

Notes:

[7] - Full Analysis Set

[8] - Full Analysis Set

[9] - Full Analysis Set

[10] - Full Analysis Set

<b>End point values</b>	Melanoma 400 mg BID Intermittent Dose			
Subject group type	Reporting group			
Number of subjects analysed	1 <sup>[11]</sup>			
Units: percentage of participants				
number (confidence interval 95%)	100.0 (2.5 to 100.0)			

Notes:

[11] - Full Analysis Set

## Statistical analyses

No statistical analyses for this end point

## Secondary: Duration of Response (DOR)

End point title	Duration of Response (DOR)
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End point description:

DOR was defined as the time from the earliest date of CR or PR, confirmed by  $\geq 1$  repeat assessment  $\geq 28$  days later, until the earliest date of disease progression by investigator assessment per RECIST v1.1, or death due to any cause, if occurring sooner than progression. CR: disappearance of all target and non-target lesions and no appearance of any new lesions. Any pathological lymph nodes (whether target or non-target) must have a reduction in the short axis to  $<10$  mm. PR: at least a 30% decrease in the sum of the diameters of target lesions, taking as a reference the baseline sum diameters, no new lesions, and no progression of non-target lesions. PD: progression of a target or non-target lesion or presence of a new lesion. 9999=Standard deviation cannot be reported for a single participant.

End point type	Secondary
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End point timeframe:

up to 733 days

<b>End point values</b>	NSCLC 400 mg BID	UC 400 mg BID	RCC 400 mg BID	Melanoma 400 mg BID
Subject group type	Reporting group	Reporting group	Reporting group	Reporting group
Number of subjects analysed	0 <sup>[12]</sup>	1 <sup>[13]</sup>	0 <sup>[14]</sup>	1 <sup>[15]</sup>
Units: months				
arithmetic mean (standard deviation)	()	3.7 ( $\pm$ 9999)	()	7.4 ( $\pm$ 9999)

Notes:

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

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

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

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

<b>End point values</b>	Melanoma 400 mg BID Intermittent Dose			
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Subject group type	Reporting group			
Number of subjects analysed	1 <sup>[16]</sup>			
Units: months				
arithmetic mean (standard deviation)	23.7 (± 9999)			

Notes:

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

## 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 considered drug related. An AE could therefore have been any unfavorable and unintended sign (including an abnormal laboratory finding), symptom, or disease (new or exacerbated) temporally associated with the use of study treatment. A TEAE was defined as any AE either reported for the first time or the worsening of a pre-existing event after the first dose of study drug up to 90 days after the last dose of study drug or until the start of new anticancer therapy, whichever occurred first.

End point type	Secondary
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End point timeframe:

up to 823 days

End point values	NSCLC 400 mg BID	UC 400 mg BID	RCC 400 mg BID	Melanoma 400 mg BID
Subject group type	Reporting group	Reporting group	Reporting group	Reporting group
Number of subjects analysed	1 <sup>[17]</sup>	1 <sup>[18]</sup>	6 <sup>[19]</sup>	7 <sup>[20]</sup>
Units: participants	1	1	6	5

Notes:

[17] - Safety Evaluable Population: all participants who received ≥1 dose of study drug.

[18] - Safety Evaluable Population: all participants who received ≥1 dose of study drug.

[19] - Safety Evaluable Population: all participants who received ≥1 dose of study drug.

[20] - Safety Evaluable Population: all participants who received ≥1 dose of study drug.

End point values	Melanoma 400 mg BID Intermittent Dose			
Subject group type	Reporting group			
Number of subjects analysed	1 <sup>[21]</sup>			
Units: participants	1			

Notes:

[21] - Safety Evaluable Population: all participants who received ≥1 dose of study drug.

## Statistical analyses

No statistical analyses for this end point

## Secondary: Number of participants with any ≥Grade 3 TEAE

End point title	Number of participants with any ≥Grade 3 TEAE
End point description: A TEAE was defined as any AE either reported for the first time or the worsening of a pre-existing event after the first dose of study drug up to 90 days after the last dose of study drug or until the start of new anticancer therapy, whichever occurred first. The severity of AEs was assessed using Common Terminology Criteria for Adverse Events version 5 (CTCAE v5) 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 823 days	

End point values	NSCLC 400 mg BID	UC 400 mg BID	RCC 400 mg BID	Melanoma 400 mg BID
Subject group type	Reporting group	Reporting group	Reporting group	Reporting group
Number of subjects analysed	1 <sup>[22]</sup>	1 <sup>[23]</sup>	6 <sup>[24]</sup>	7 <sup>[25]</sup>
Units: participants	0	1	3	0

Notes:

[22] - Safety Evaluable Population

[23] - Safety Evaluable Population

[24] - Safety Evaluable Population

[25] - Safety Evaluable Population

End point values	Melanoma 400 mg BID Intermittent Dose			
Subject group type	Reporting group			
Number of subjects analysed	1 <sup>[26]</sup>			
Units: participants	0			

Notes:

[26] - Safety Evaluable Population

## Statistical analyses

No statistical analyses for this end point

## Adverse events

### Adverse events information

Timeframe for reporting adverse events:

from the time of Informed Consent Form signing until at least 90 days after the last dose of study drug or until the start of new anticancer therapy, whichever occurred first (up to approximately 2.5 years)

Adverse event reporting additional description:

Adverse events were assessed in members of the Safety Evaluable Population, comprised of all participants who received received  $\geq 1$  dose of study drug.

Assessment type	Systematic
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### Dictionary used

Dictionary name	MedDRA
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Dictionary version	26.1
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### Reporting groups

Reporting group title	NSCLC 400 mg BID
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Reporting group description:

Participants with non-small cell lung cancer (NSCLC) received INCB086550 orally in 28-day cycles at a dose of 400 milligrams (mg) twice daily (BID) for up to 2 years as long as they received benefit and didn't meet any criteria for study withdrawal. Participants who achieved a complete response could have continued to receive INCB086550 for an additional 4 cycles (with a minimum of 1 year of treatment) upon medical monitor consultation.

Reporting group title	UC 400 mg BID
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Reporting group description:

Participants with urothelial carcinoma (UC) received INCB086550 orally in 28-day cycles at a dose of 400 mg BID for up to 2 years as long as they received benefit and didn't meet any criteria for study withdrawal. Participants who achieved a complete response could have continued to receive INCB086550 for an additional 4 cycles (with a minimum of 1 year of treatment) upon medical monitor consultation.

Reporting group title	RCC 400 mg BID
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Reporting group description:

Participants with renal cell carcinoma (RC) received INCB086550 orally in 28-day cycles at a dose of 400 mg BID for up to 2 years as long as they received benefit and didn't meet any criteria for study withdrawal. Participants who achieved a complete response could have continued to receive INCB086550 for an additional 4 cycles (with a minimum of 1 year of treatment) upon medical monitor consultation.

Reporting group title	Melanoma 400 mg BID
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Reporting group description:

Participants with melanoma received INCB086550 orally in 28-day cycles at a dose of 400 mg BID for up to 2 years as long as they received benefit and didn't meet any criteria for study withdrawal. Participants who achieved a complete response could have continued to receive INCB086550 for an additional 4 cycles (with a minimum of 1 year of treatment) upon medical monitor consultation.

Reporting group title	Melanoma 400 mg BID Intermittent Dose
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Reporting group description:

Participants with melanoma received intermittent INCB086550 orally in 28-day cycles at a dose of 400 mg BID. Participants received INCB086550 for 1 week followed by 1 week off for up to 2 years as long as they received benefit and didn't meet any criteria for study withdrawal. Participants who achieved a complete response could have continued to receive INCB086550 for an additional 4 cycles (with a minimum of 1 year of treatment) upon medical monitor consultation.

Reporting group title	Total
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Reporting group description:

Total

<b>Serious adverse events</b>	NSCLC 400 mg BID	UC 400 mg BID	RCC 400 mg BID
Total subjects affected by serious adverse events			
subjects affected / exposed	0 / 1 (0.00%)	1 / 1 (100.00%)	2 / 6 (33.33%)
number of deaths (all causes)	1	0	2
number of deaths resulting from adverse events	0	0	0
Nervous system disorders			
Immune-mediated neuropathy			
subjects affected / exposed	0 / 1 (0.00%)	1 / 1 (100.00%)	0 / 6 (0.00%)
occurrences causally related to treatment / all	0 / 0	1 / 1	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Blood and lymphatic system disorders			
Iron deficiency anaemia			
subjects affected / exposed	0 / 1 (0.00%)	0 / 1 (0.00%)	1 / 6 (16.67%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Infections and infestations			
Pneumonia			
subjects affected / exposed	0 / 1 (0.00%)	0 / 1 (0.00%)	1 / 6 (16.67%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Metabolism and nutrition disorders			
Hyponatraemia			
subjects affected / exposed	0 / 1 (0.00%)	0 / 1 (0.00%)	1 / 6 (16.67%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0

<b>Serious adverse events</b>	Melanoma 400 mg BID	Melanoma 400 mg BID Intermittent Dose	Total
Total subjects affected by serious adverse events			
subjects affected / exposed	0 / 7 (0.00%)	0 / 1 (0.00%)	3 / 16 (18.75%)
number of deaths (all causes)	2	0	5
number of deaths resulting from adverse events	0	0	0
Nervous system disorders			
Immune-mediated neuropathy			
subjects affected / exposed	0 / 7 (0.00%)	0 / 1 (0.00%)	1 / 16 (6.25%)
occurrences causally related to treatment / all	0 / 0	0 / 0	1 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0

Blood and lymphatic system disorders			
Iron deficiency anaemia			
subjects affected / exposed	0 / 7 (0.00%)	0 / 1 (0.00%)	1 / 16 (6.25%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Infections and infestations			
Pneumonia			
subjects affected / exposed	0 / 7 (0.00%)	0 / 1 (0.00%)	1 / 16 (6.25%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Metabolism and nutrition disorders			
Hyponatraemia			
subjects affected / exposed	0 / 7 (0.00%)	0 / 1 (0.00%)	1 / 16 (6.25%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 1
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>	<b>NSCLC 400 mg BID</b>	<b>UC 400 mg BID</b>	<b>RCC 400 mg BID</b>
Total subjects affected by non-serious adverse events			
subjects affected / exposed	1 / 1 (100.00%)	1 / 1 (100.00%)	6 / 6 (100.00%)
Neoplasms benign, malignant and unspecified (incl cysts and polyps)			
Cancer pain			
subjects affected / exposed	0 / 1 (0.00%)	0 / 1 (0.00%)	0 / 6 (0.00%)
occurrences (all)	0	0	0
Cerebral haemangioma			
subjects affected / exposed	0 / 1 (0.00%)	0 / 1 (0.00%)	1 / 6 (16.67%)
occurrences (all)	0	0	1
Haemangioma			
subjects affected / exposed	0 / 1 (0.00%)	0 / 1 (0.00%)	1 / 6 (16.67%)
occurrences (all)	0	0	1
Uterine leiomyoma			
subjects affected / exposed	0 / 1 (0.00%)	0 / 1 (0.00%)	0 / 6 (0.00%)
occurrences (all)	0	0	0
Vascular disorders			



Arteriovenous fistula subjects affected / exposed occurrences (all)	0 / 1 (0.00%) 0	0 / 1 (0.00%) 0	1 / 6 (16.67%) 1
General disorders and administration site conditions			
Asthenia subjects affected / exposed occurrences (all)	0 / 1 (0.00%) 0	0 / 1 (0.00%) 0	0 / 6 (0.00%) 0
Fatigue subjects affected / exposed occurrences (all)	0 / 1 (0.00%) 0	0 / 1 (0.00%) 0	2 / 6 (33.33%) 3
Oedema peripheral subjects affected / exposed occurrences (all)	0 / 1 (0.00%) 0	0 / 1 (0.00%) 0	1 / 6 (16.67%) 1
Immune system disorders			
Hypersensitivity subjects affected / exposed occurrences (all)	0 / 1 (0.00%) 0	0 / 1 (0.00%) 0	0 / 6 (0.00%) 0
Respiratory, thoracic and mediastinal disorders			
Cough subjects affected / exposed occurrences (all)	0 / 1 (0.00%) 0	0 / 1 (0.00%) 0	2 / 6 (33.33%) 2
Investigations			
Aspartate aminotransferase increased subjects affected / exposed occurrences (all)	0 / 1 (0.00%) 0	0 / 1 (0.00%) 0	1 / 6 (16.67%) 3
Alanine aminotransferase increased subjects affected / exposed occurrences (all)	0 / 1 (0.00%) 0	0 / 1 (0.00%) 0	1 / 6 (16.67%) 2
Blood alkaline phosphatase increased subjects affected / exposed occurrences (all)	0 / 1 (0.00%) 0	0 / 1 (0.00%) 0	0 / 6 (0.00%) 0
Electrocardiogram QT prolonged subjects affected / exposed occurrences (all)	0 / 1 (0.00%) 0	0 / 1 (0.00%) 0	1 / 6 (16.67%) 1
Lipase increased			

subjects affected / exposed occurrences (all)	0 / 1 (0.00%) 0	0 / 1 (0.00%) 0	0 / 6 (0.00%) 0
Weight decreased subjects affected / exposed occurrences (all)	0 / 1 (0.00%) 0	0 / 1 (0.00%) 0	1 / 6 (16.67%) 2
Cardiac disorders Sinus bradycardia subjects affected / exposed occurrences (all)	0 / 1 (0.00%) 0	0 / 1 (0.00%) 0	1 / 6 (16.67%) 1
Nervous system disorders Brain compression subjects affected / exposed occurrences (all)	0 / 1 (0.00%) 0	0 / 1 (0.00%) 0	1 / 6 (16.67%) 1
Headache subjects affected / exposed occurrences (all)	0 / 1 (0.00%) 0	0 / 1 (0.00%) 0	1 / 6 (16.67%) 2
Hypoaesthesia subjects affected / exposed occurrences (all)	0 / 1 (0.00%) 0	0 / 1 (0.00%) 0	0 / 6 (0.00%) 0
Immune-mediated neuropathy subjects affected / exposed occurrences (all)	0 / 1 (0.00%) 0	1 / 1 (100.00%) 2	0 / 6 (0.00%) 0
Peripheral sensory neuropathy subjects affected / exposed occurrences (all)	0 / 1 (0.00%) 0	0 / 1 (0.00%) 0	1 / 6 (16.67%) 1
Blood and lymphatic system disorders Anaemia subjects affected / exposed occurrences (all)	0 / 1 (0.00%) 0	0 / 1 (0.00%) 0	3 / 6 (50.00%) 3
Thrombocytopenia subjects affected / exposed occurrences (all)	0 / 1 (0.00%) 0	0 / 1 (0.00%) 0	0 / 6 (0.00%) 0
Ear and labyrinth disorders Vertigo subjects affected / exposed occurrences (all)	0 / 1 (0.00%) 0	0 / 1 (0.00%) 0	1 / 6 (16.67%) 1
Gastrointestinal disorders			

Gastritis			
subjects affected / exposed	0 / 1 (0.00%)	0 / 1 (0.00%)	0 / 6 (0.00%)
occurrences (all)	0	0	0
Nausea			
subjects affected / exposed	0 / 1 (0.00%)	0 / 1 (0.00%)	2 / 6 (33.33%)
occurrences (all)	0	0	4
Skin and subcutaneous tissue disorders			
Pruritus			
subjects affected / exposed	0 / 1 (0.00%)	0 / 1 (0.00%)	1 / 6 (16.67%)
occurrences (all)	0	0	1
Rash			
subjects affected / exposed	0 / 1 (0.00%)	0 / 1 (0.00%)	1 / 6 (16.67%)
occurrences (all)	0	0	1
Musculoskeletal and connective tissue disorders			
Arthralgia			
subjects affected / exposed	0 / 1 (0.00%)	0 / 1 (0.00%)	1 / 6 (16.67%)
occurrences (all)	0	0	2
Back pain			
subjects affected / exposed	0 / 1 (0.00%)	0 / 1 (0.00%)	2 / 6 (33.33%)
occurrences (all)	0	0	2
Ligamentum flavum hypertrophy			
subjects affected / exposed	0 / 1 (0.00%)	0 / 1 (0.00%)	1 / 6 (16.67%)
occurrences (all)	0	0	1
Intervertebral disc protrusion			
subjects affected / exposed	0 / 1 (0.00%)	0 / 1 (0.00%)	1 / 6 (16.67%)
occurrences (all)	0	0	1
Myalgia			
subjects affected / exposed	0 / 1 (0.00%)	0 / 1 (0.00%)	1 / 6 (16.67%)
occurrences (all)	0	0	1
Osteoporosis			
subjects affected / exposed	0 / 1 (0.00%)	0 / 1 (0.00%)	1 / 6 (16.67%)
occurrences (all)	0	0	1
Osteochondrosis			
subjects affected / exposed	0 / 1 (0.00%)	0 / 1 (0.00%)	1 / 6 (16.67%)
occurrences (all)	0	0	1
Neck pain			

subjects affected / exposed	0 / 1 (0.00%)	0 / 1 (0.00%)	0 / 6 (0.00%)
occurrences (all)	0	0	0
Pain in extremity			
subjects affected / exposed	1 / 1 (100.00%)	0 / 1 (0.00%)	1 / 6 (16.67%)
occurrences (all)	1	0	1
Scoliosis			
subjects affected / exposed	0 / 1 (0.00%)	0 / 1 (0.00%)	1 / 6 (16.67%)
occurrences (all)	0	0	1
Spinal osteoarthritis			
subjects affected / exposed	0 / 1 (0.00%)	0 / 1 (0.00%)	1 / 6 (16.67%)
occurrences (all)	0	0	1
Infections and infestations			
Coronavirus infection			
subjects affected / exposed	0 / 1 (0.00%)	1 / 1 (100.00%)	0 / 6 (0.00%)
occurrences (all)	0	1	0
COVID-19			
subjects affected / exposed	0 / 1 (0.00%)	0 / 1 (0.00%)	1 / 6 (16.67%)
occurrences (all)	0	0	1
Cystitis			
subjects affected / exposed	0 / 1 (0.00%)	0 / 1 (0.00%)	1 / 6 (16.67%)
occurrences (all)	0	0	1
Pneumonia			
subjects affected / exposed	0 / 1 (0.00%)	0 / 1 (0.00%)	1 / 6 (16.67%)
occurrences (all)	0	0	3
Pyelonephritis chronic			
subjects affected / exposed	0 / 1 (0.00%)	0 / 1 (0.00%)	1 / 6 (16.67%)
occurrences (all)	0	0	1
Metabolism and nutrition disorders			
Hyperkalaemia			
subjects affected / exposed	1 / 1 (100.00%)	0 / 1 (0.00%)	0 / 6 (0.00%)
occurrences (all)	2	0	0
Decreased appetite			
subjects affected / exposed	0 / 1 (0.00%)	0 / 1 (0.00%)	1 / 6 (16.67%)
occurrences (all)	0	0	1
Hypocalcaemia			

subjects affected / exposed	0 / 1 (0.00%)	0 / 1 (0.00%)	1 / 6 (16.67%)
occurrences (all)	0	0	1

<b>Non-serious adverse events</b>	Melanoma 400 mg BID	Melanoma 400 mg BID Intermittent Dose	Total
Total subjects affected by non-serious adverse events			
subjects affected / exposed	5 / 7 (71.43%)	1 / 1 (100.00%)	14 / 16 (87.50%)
Neoplasms benign, malignant and unspecified (incl cysts and polyps)			
Cancer pain			
subjects affected / exposed	1 / 7 (14.29%)	0 / 1 (0.00%)	1 / 16 (6.25%)
occurrences (all)	1	0	1
Cerebral haemangioma			
subjects affected / exposed	0 / 7 (0.00%)	0 / 1 (0.00%)	1 / 16 (6.25%)
occurrences (all)	0	0	1
Haemangioma			
subjects affected / exposed	0 / 7 (0.00%)	0 / 1 (0.00%)	1 / 16 (6.25%)
occurrences (all)	0	0	1
Uterine leiomyoma			
subjects affected / exposed	0 / 7 (0.00%)	1 / 1 (100.00%)	1 / 16 (6.25%)
occurrences (all)	0	1	1
Vascular disorders			
Arteriovenous fistula			
subjects affected / exposed	0 / 7 (0.00%)	0 / 1 (0.00%)	1 / 16 (6.25%)
occurrences (all)	0	0	1
General disorders and administration site conditions			
Asthenia			
subjects affected / exposed	2 / 7 (28.57%)	0 / 1 (0.00%)	2 / 16 (12.50%)
occurrences (all)	4	0	4
Fatigue			
subjects affected / exposed	0 / 7 (0.00%)	0 / 1 (0.00%)	2 / 16 (12.50%)
occurrences (all)	0	0	3
Oedema peripheral			
subjects affected / exposed	0 / 7 (0.00%)	0 / 1 (0.00%)	1 / 16 (6.25%)
occurrences (all)	0	0	1
Immune system disorders			

Hypersensitivity subjects affected / exposed occurrences (all)	1 / 7 (14.29%) 1	0 / 1 (0.00%) 0	1 / 16 (6.25%) 1
Respiratory, thoracic and mediastinal disorders Cough subjects affected / exposed occurrences (all)	0 / 7 (0.00%) 0	0 / 1 (0.00%) 0	2 / 16 (12.50%) 2
Investigations Aspartate aminotransferase increased subjects affected / exposed occurrences (all)	1 / 7 (14.29%) 1	0 / 1 (0.00%) 0	2 / 16 (12.50%) 4
Alanine aminotransferase increased subjects affected / exposed occurrences (all)	1 / 7 (14.29%) 1	0 / 1 (0.00%) 0	2 / 16 (12.50%) 3
Blood alkaline phosphatase increased subjects affected / exposed occurrences (all)	1 / 7 (14.29%) 2	0 / 1 (0.00%) 0	1 / 16 (6.25%) 2
Electrocardiogram QT prolonged subjects affected / exposed occurrences (all)	0 / 7 (0.00%) 0	0 / 1 (0.00%) 0	1 / 16 (6.25%) 1
Lipase increased subjects affected / exposed occurrences (all)	1 / 7 (14.29%) 1	0 / 1 (0.00%) 0	1 / 16 (6.25%) 1
Weight decreased subjects affected / exposed occurrences (all)	0 / 7 (0.00%) 0	0 / 1 (0.00%) 0	1 / 16 (6.25%) 2
Cardiac disorders Sinus bradycardia subjects affected / exposed occurrences (all)	0 / 7 (0.00%) 0	0 / 1 (0.00%) 0	1 / 16 (6.25%) 1
Nervous system disorders Brain compression subjects affected / exposed occurrences (all)	0 / 7 (0.00%) 0	0 / 1 (0.00%) 0	1 / 16 (6.25%) 1
Headache			

subjects affected / exposed occurrences (all)	2 / 7 (28.57%) 4	0 / 1 (0.00%) 0	3 / 16 (18.75%) 6
Hypoaesthesia subjects affected / exposed occurrences (all)	1 / 7 (14.29%) 1	0 / 1 (0.00%) 0	1 / 16 (6.25%) 1
Immune-mediated neuropathy subjects affected / exposed occurrences (all)	0 / 7 (0.00%) 0	0 / 1 (0.00%) 0	1 / 16 (6.25%) 2
Peripheral sensory neuropathy subjects affected / exposed occurrences (all)	0 / 7 (0.00%) 0	1 / 1 (100.00%) 1	2 / 16 (12.50%) 2
Blood and lymphatic system disorders Anaemia subjects affected / exposed occurrences (all)	1 / 7 (14.29%) 1	0 / 1 (0.00%) 0	4 / 16 (25.00%) 4
Thrombocytopenia subjects affected / exposed occurrences (all)	0 / 7 (0.00%) 0	1 / 1 (100.00%) 1	1 / 16 (6.25%) 1
Ear and labyrinth disorders Vertigo subjects affected / exposed occurrences (all)	0 / 7 (0.00%) 0	0 / 1 (0.00%) 0	1 / 16 (6.25%) 1
Gastrointestinal disorders Gastritis subjects affected / exposed occurrences (all)	1 / 7 (14.29%) 1	0 / 1 (0.00%) 0	1 / 16 (6.25%) 1
Nausea subjects affected / exposed occurrences (all)	0 / 7 (0.00%) 0	0 / 1 (0.00%) 0	2 / 16 (12.50%) 4
Skin and subcutaneous tissue disorders Pruritus subjects affected / exposed occurrences (all)	1 / 7 (14.29%) 1	0 / 1 (0.00%) 0	2 / 16 (12.50%) 2
Rash subjects affected / exposed occurrences (all)	0 / 7 (0.00%) 0	0 / 1 (0.00%) 0	1 / 16 (6.25%) 1
Musculoskeletal and connective tissue			

disorders			
Arthralgia			
subjects affected / exposed	0 / 7 (0.00%)	0 / 1 (0.00%)	1 / 16 (6.25%)
occurrences (all)	0	0	2
Back pain			
subjects affected / exposed	0 / 7 (0.00%)	0 / 1 (0.00%)	2 / 16 (12.50%)
occurrences (all)	0	0	2
Ligamentum flavum hypertrophy			
subjects affected / exposed	0 / 7 (0.00%)	0 / 1 (0.00%)	1 / 16 (6.25%)
occurrences (all)	0	0	1
Intervertebral disc protrusion			
subjects affected / exposed	0 / 7 (0.00%)	0 / 1 (0.00%)	1 / 16 (6.25%)
occurrences (all)	0	0	1
Myalgia			
subjects affected / exposed	0 / 7 (0.00%)	0 / 1 (0.00%)	1 / 16 (6.25%)
occurrences (all)	0	0	1
Osteoporosis			
subjects affected / exposed	0 / 7 (0.00%)	0 / 1 (0.00%)	1 / 16 (6.25%)
occurrences (all)	0	0	1
Osteochondrosis			
subjects affected / exposed	0 / 7 (0.00%)	0 / 1 (0.00%)	1 / 16 (6.25%)
occurrences (all)	0	0	1
Neck pain			
subjects affected / exposed	1 / 7 (14.29%)	0 / 1 (0.00%)	1 / 16 (6.25%)
occurrences (all)	1	0	1
Pain in extremity			
subjects affected / exposed	1 / 7 (14.29%)	0 / 1 (0.00%)	3 / 16 (18.75%)
occurrences (all)	4	0	6
Scoliosis			
subjects affected / exposed	0 / 7 (0.00%)	0 / 1 (0.00%)	1 / 16 (6.25%)
occurrences (all)	0	0	1
Spinal osteoarthritis			
subjects affected / exposed	0 / 7 (0.00%)	0 / 1 (0.00%)	1 / 16 (6.25%)
occurrences (all)	0	0	1
Infections and infestations			



Coronavirus infection subjects affected / exposed occurrences (all)	0 / 7 (0.00%) 0	0 / 1 (0.00%) 0	1 / 16 (6.25%) 1
COVID-19 subjects affected / exposed occurrences (all)	2 / 7 (28.57%) 2	0 / 1 (0.00%) 0	3 / 16 (18.75%) 3
Cystitis subjects affected / exposed occurrences (all)	0 / 7 (0.00%) 0	0 / 1 (0.00%) 0	1 / 16 (6.25%) 1
Pneumonia subjects affected / exposed occurrences (all)	0 / 7 (0.00%) 0	0 / 1 (0.00%) 0	1 / 16 (6.25%) 3
Pyelonephritis chronic subjects affected / exposed occurrences (all)	0 / 7 (0.00%) 0	0 / 1 (0.00%) 0	1 / 16 (6.25%) 1
Metabolism and nutrition disorders			
Hyperkalaemia subjects affected / exposed occurrences (all)	0 / 7 (0.00%) 0	0 / 1 (0.00%) 0	1 / 16 (6.25%) 2
Decreased appetite subjects affected / exposed occurrences (all)	0 / 7 (0.00%) 0	0 / 1 (0.00%) 0	1 / 16 (6.25%) 1
Hypocalcaemia subjects affected / exposed occurrences (all)	0 / 7 (0.00%) 0	0 / 1 (0.00%) 0	1 / 16 (6.25%) 1

## More information

### Substantial protocol amendments (globally)

Were there any global substantial amendments to the protocol? Yes

Date	Amendment
08 March 2021	The primary purpose of this amendment was to incorporate additional dose levels to evaluate the safety and preliminary activity of INCB086550 with intermittent and/or step-down dose administration schedules.

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.

Enrollment in this study was discontinued after INCB086550 program development was terminated by the sponsor due to business reasons.
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Notes: