Clinical trial results:
A Phase II open-label, single-arm, multi-center study of ruxolitinib added to corticosteroids in pediatric subjects with moderate and severe chronic graft vs. host disease after allogeneic stem cell transplantation – final analysis

Due to EudraCT system limitations, which EMA is aware of, data using 999 as data points in this record are not an accurate representation of the clinical trial results.
Please use https://www.novctrd.com for complete trial results.

Summary

EudraCT number	2018-003296-35
Trial protocol	SK IT CZ LT GR ES SI
Global end of trial date	26 August 2024

Results information

Result version number	v2 (current)
This version publication date	17 May 2025
First version publication date	15 March 2025
Version creation reason	

Trial information

Trial identification

Sponsor protocol code	CINC424G12201

Additional study identifiers

ISRCTN number	-
ClinicalTrials.gov id (NCT number)	NCT03774082
WHO universal trial number (UTN)	-

Notes:

Sponsors

Sponsor organisation name	Novartis Pharma, AG
Sponsor organisation address	CH-4002, Basel, Switzerland,
Public contact	Clinical Disclosure Office, Novartis Pharma, AG, 41 613241111, novatis.email@novartis.com
Scientific contact	Clinical Disclosure Office, Novartis Pharma, AG, 41 613241111, novatis.email@novartis.com

Notes:

Paediatric regulatory details

Is trial part of an agreed paediatric	Yes

investigation plan (PIP)	
EMA paediatric investigation plan number(s)	EMEA-000901-PIP04-17
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?	Yes

Notes:

Results analysis stage

Analysis stage	Final
Date of interim/final analysis	25 February 2022
Is this the analysis of the primary completion data?	No

Global end of trial reached?	Yes
Global end of trial date	26 August 2024
Was the trial ended prematurely?	No

Notes:

General information about the trial

Main objective of the trial:

The primary objective of this study was to evaluate the activity of ruxolitinib added to standard dose corticosteroids, ± calcineurin inhibitor (CNI), in pediatric subjects with moderate or severe treatment-naïve chronic GvHD or steroid-refractory chronic GvHD, by measuring the overall response rate (ORR) at Cycle 7 Day 1, based on all subjects in the study. ORR was defined as the proportion of subjects demonstrating a complete response (CR) or partial response (PR) without the requirement of additional systemic therapies for an earlier progression, mixed response or non-response.

Protection of trial subjects:

The study was in compliance with the ethical principles derived from the Declaration of Helsinki and the International Conference on Harmonization (ICH) Good Clinical Practice (GCP) guidelines. All the local regulatory requirements pertinent to safety of trial subjects were also followed during the conduct of the trial.

Background therapy: -

Evidence for comparator: -

Actual start date of recruitment	20 May 2020
Long term follow-up planned	No
Independent data monitoring committee (IDMC) involvement?	Yes

Notes:

Population of trial subjects

Subjects enrolled per country

Country: Number of subjects enrolled	Brazil: 3
Country: Number of subjects enrolled	Canada: 1
Country: Number of subjects enrolled	Czechia: 1
Country: Number of subjects enrolled	India: 6
Country: Number of subjects enrolled	Italy: 3
Country: Number of subjects enrolled	Japan: 7
Country: Number of subjects enrolled	Korea, Republic of: 6
Country: Number of subjects enrolled	Russian Federation: 1

Country: Number of subjects enrolled	Slovakia: 1
Country: Number of subjects enrolled	Spain: 2
Country: Number of subjects enrolled	Switzerland: 2
Country: Number of subjects enrolled	Taiwan: 2
Country: Number of subjects enrolled	Thailand: 1
Country: Number of subjects enrolled	Türkiye: 10
Worldwide total number of subjects	46
EEA total number of subjects	7

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)	24
Adolescents (12-17 years)	22
Adults (18-64 years)	0
From 65 to 84 years	0
85 years and over	0

Subject disposition

Recruitment

Recruitment details:

At least 5 evaluable participants per group were needed for the primary analysis in Groups 1, 2 and 3. No minimum number of evaluable participants were needed in Group 4.
Disposition and Demographics are presented by age group.

Pre-assignment

Screening details:

Enrollment initiation into the youngest age group, Group 4, was subject to the availability of data in this age group from study CINC424F12201, as well as a review of available PK, safety, and activity data generated from Groups 1 to 3 in the current study.

Period 1

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

Arms

Are arms mutually exclusive?	Yes
Arm title	≥ 12y - < 18y RUX 10mg BID (Group 1)

Arm description:

Participants received ruxolitinib 10mg orally twice a day (BID).

Arm type	Experimental
Investigational medicinal product name	Ruxolitinib
Investigational medicinal product code	INC424
Other name	
Pharmaceutical forms	Tablet
Routes of administration	Oral use

Dosage and administration details:

10mg ruxolitinib was taken twice a day (BID)

Arm title	≥ 6y - < 12y RUX 5mg BID (Group 2)

Arm description:

Participants received ruxolitinib 5mg orally twice a day (BID).

Arm type	Experimental
Investigational medicinal product name	Ruxolitinib
Investigational medicinal product code	INC424
Other name	
Pharmaceutical forms	Tablet, Oral liquid
Routes of administration	Oral use

Dosage and administration details:

5mg ruxolitinib was taken twice a day (BID) as tablet or liquid

Arm title	≥ 2y - < 6y RUX 4mg/m2 BID (Group 3)

Arm description:

Participants received ruxolitinib 4mg/m2 orally twice a day (BID).

Arm type	Experimental

Investigational medicinal product name	Ruxolitinib
Investigational medicinal product code	INC424
Other name	
Pharmaceutical forms	Oral liquid
Routes of administration	Oral use

Dosage and administration details:

4mg/m2 ruxolitinib was taken twice a day (BID) as liquid

Number of subjects in period 1	≥ 12y - < 18y RUX 10mg BID (Group 1)	≥ 6y - < 12y RUX 5mg BID (Group 2)	≥ 2y - < 6y RUX 4mg/m2 BID (Group 3)
Started	22	17	7
Full Analysis Set	22	16	7
Completed treatment	3 [1]	5 [2]	3 [3]
Discontinued from treatment	19	11	4
Subjects who received tablets	22	14	0 [4]
Subjects who received liquid	0 [5]	2 [6]	7
Completed	14	10	4
Not completed	8	7	3
Adverse event, serious fatal	6	3	2
Subject decision	-	3	-
Administrative reasons	-	1	-
Lost to follow-up	2	-	-
Guardian decision	-	-	1

Notes:

[1] - The number of subjects at this milestone seems inconsistent with the number of subjects in the arm. It is expected that the number of subjects will be greater than, or equal to the number that completed, minus those who left.
Justification: This is correct as is. These subjects here completed treatment, not study.

[2] - The number of subjects at this milestone seems inconsistent with the number of subjects in the arm. It is expected that the number of subjects will be greater than, or equal to the number that completed, minus those who left.
Justification: Milestone added for clarification purposes only.

[3] - The number of subjects at this milestone seems inconsistent with the number of subjects in the arm. It is expected that the number of subjects will be greater than, or equal to the number that completed, minus those who left.
Justification: Milestone added for clarification purposes only.

[4] - The number of subjects at this milestone seems inconsistent with the number of subjects in the arm. It is expected that the number of subjects will be greater than, or equal to the number that completed, minus those who left.
Justification: This is correct as is. These subjects here completed treatment, not study.

[5] - The number of subjects at this milestone seems inconsistent with the number of subjects in the arm. It is expected that the number of subjects will be greater than, or equal to the number that completed, minus those who left.
Justification: Milestone added for clarification purposes only.

[6] - The number of subjects at this milestone seems inconsistent with the number of subjects in the arm. It is expected that the number of subjects will be greater than, or equal to the number that completed, minus those who left.
Justification: This is correct as is. These subjects here completed treatment, not study.

Baseline characteristics

Reporting groups	
Reporting group title	≥ 12y - < 18y RUX 10mg BID (Group 1)
Reporting group description:	
Participants received ruxolitinib 10mg orally twice a day (BID).	
Reporting group title	≥ 6y - < 12y RUX 5mg BID (Group 2)
Reporting group description:	
Participants received ruxolitinib 5mg orally twice a day (BID).	
Reporting group title	≥ 2y - < 6y RUX 4mg/m2 BID (Group 3)
Reporting group description:	
Participants received ruxolitinib 4mg/m2 orally twice a day (BID).	

Reporting group values	≥ 12y - < 18y RUX 10mg BID (Group 1)	≥ 6y - < 12y RUX 5mg BID (Group 2)	≥ 2y - < 6y RUX 4mg/m2 BID (Group 3)
Number of subjects	22	17	7
Age categorical Units: Subjects			
Children (2-11 years)	0	17	7
Adolescents (12-17 years)	22	0	0
Age Continuous Units: months			
arithmetic mean	175.2	99.3	47.1
standard deviation	± 20.05	± 18.39	± 15.08
Sex: Female, Male Units: Participants			
Female	7	8	2
Male	15	9	5
Race/Ethnicity, Customized Units: Subjects			
White	8	11	3
Black or African American	0	1	0
Asian	14	5	4

Reporting group values	Total		
Number of subjects	46		
Age categorical Units: Subjects			
Children (2-11 years)	24		
Adolescents (12-17 years)	22		
Age Continuous Units: months			
arithmetic mean			
standard deviation	-		
Sex: Female, Male Units: Participants			
Female	17		
Male	29		

Race/Ethnicity, Customized Units: Subjects			
White	22		
Black or African American	1		
Asian	23		

Subject analysis sets

Subject analysis set title	All Participants
Subject analysis set type	Full analysis
Subject analysis set description: All participants from Group 1, Group 2 and Group 3.	
Subject analysis set title	≥ 12y - < 18y RUX 10mg BID (Tablet)
Subject analysis set type	Sub-group analysis
Subject analysis set description: Participants in this group were administered 10mg ruxolitinib tablet twice a day.	
Subject analysis set title	≥ 6y - < 12y RUX 5mg BID (Tablet)
Subject analysis set type	Sub-group analysis
Subject analysis set description: Participants in this group were administered 5mg ruxolitinib tablets twice a day.	
Subject analysis set title	≥ 6y - < 12y RUX 5mg BID (Liquid)
Subject analysis set type	Sub-group analysis
Subject analysis set description: Participants in this group were administered ruxolitinib oral pediatric formulation twice a day.	
Subject analysis set title	≥ 2y - < 6y RUX 4mg/m2 BID (Liquid)
Subject analysis set type	Sub-group analysis
Subject analysis set description: Participants in this group were administered ruxolitinib oral pediatric formulation twice a day.	
Subject analysis set title	All Participants
Subject analysis set type	Sub-group analysis
Subject analysis set description: DOR was analyzed for all responders from the three age groups (Groups 1, 2 and 3)	
Subject analysis set title	All Participants
Subject analysis set type	Full analysis
Subject analysis set description: FFS was analyzed for all participants from the three age groups (Groups 1, 2 and 3)	
Subject analysis set title	All Participants
Subject analysis set type	Sub-group analysis
Subject analysis set description: MR was analyzed for all participants with underlying hematologic malignant disease from the three age groups (Groups 1, 2 and 3).	
Subject analysis set title	All Participants
Subject analysis set type	Full analysis
Subject analysis set description: NRM was analyzed for all participants from the three age groups (Groups 1, 2 and 3).	
Subject analysis set title	All Participants
Subject analysis set type	Full analysis
Subject analysis set description: OS was analyzed for all participants from the three age groups (Groups 1, 2 and 3).	
Subject analysis set title	All Participants
Subject analysis set type	Sub-group analysis

Subject analysis set description:

The percentage of participants with ≥50% reduction from baseline in daily corticosteroid dose at least once was analyzed for all participants in Groups 1, 2 and 3 who received corticosteroids at baseline.

Subject analysis set title	All Participants
Subject analysis set type	Sub-group analysis

Subject analysis set description:

The percentage of participants with reduction to <=0.2mg/kg/day from baseline in daily corticosteroid dose at least once was analyzed for all participants in Groups 1, 2 and 3 who received corticosteroids at baseline.

Subject analysis set title	All Participants
Subject analysis set type	Full analysis

Subject analysis set description:

Graft Failure was analyzed for all participants from the three age groups (Groups 1, 2 and 3).

Reporting group values	All Participants	≥ 12y - < 18y RUX 10mg BID (Tablet)	≥ 6y - < 12y RUX 5mg BID (Tablet)
Number of subjects	45	21	13
Age categorical Units: Subjects			
Children (2-11 years)			
Adolescents (12-17 years)			
Age Continuous Units: months			
arithmetic mean	128.3		
standard deviation	± 52.86	±	±
Sex: Female, Male Units: Participants			
Female			
Male			
Race/Ethnicity, Customized Units: Subjects			
White			
Black or African American			
Asian			

Reporting group values	≥ 6y - < 12y RUX 5mg BID (Liquid)	≥ 2y - < 6y RUX 4mg/m2 BID (Liquid)	All Participants
Number of subjects	2	7	37
Age categorical Units: Subjects			
Children (2-11 years)			
Adolescents (12-17 years)			
Age Continuous Units: months			
arithmetic mean			
standard deviation	±	±	±
Sex: Female, Male Units: Participants			
Female			
Male			

Race/Ethnicity, Customized Units: Subjects			
White Black or African American Asian			

Reporting group values	All Participants	All Participants	All Participants
Number of subjects	45	30	45
Age categorical Units: Subjects			
Children (2-11 years) Adolescents (12-17 years)			
Age Continuous Units: months arithmetic mean standard deviation	±	±	±
Sex: Female, Male Units: Participants			
Female Male			
Race/Ethnicity, Customized Units: Subjects			
White Black or African American Asian			

Reporting group values	All Participants	All Participants	All Participants
Number of subjects	45	40	40
Age categorical Units: Subjects			
Children (2-11 years) Adolescents (12-17 years)			
Age Continuous Units: months arithmetic mean standard deviation	±	±	±
Sex: Female, Male Units: Participants			
Female Male			
Race/Ethnicity, Customized Units: Subjects			
White Black or African American Asian			

Reporting group values	All Participants		
Number of subjects	45		
Age categorical Units: Subjects			
Children (2-11 years)			

Adolescents (12-17 years)			

Age Continuous Units: months arithmetic mean standard deviation	±		
Sex: Female, Male Units: Participants			
Female Male			
Race/Ethnicity, Customized Units: Subjects			
White Black or African American Asian			

End points

End points reporting groups

Reporting group title	≥ 12y - < 18y RUX 10mg BID (Group 1)
Reporting group description:	
Participants received ruxolitinib 10mg orally twice a day (BID).	
Reporting group title	≥ 6y - < 12y RUX 5mg BID (Group 2)
Reporting group description:	
Participants received ruxolitinib 5mg orally twice a day (BID).	
Reporting group title	≥ 2y - < 6y RUX 4mg/m2 BID (Group 3)
Reporting group description:	
Participants received ruxolitinib 4mg/m2 orally twice a day (BID).	
Subject analysis set title	All Participants
Subject analysis set type	Full analysis
Subject analysis set description:	
All participants from Group 1, Group 2 and Group 3.	
Subject analysis set title	≥ 12y - < 18y RUX 10mg BID (Tablet)
Subject analysis set type	Sub-group analysis
Subject analysis set description:	
Participants in this group were administered 10mg ruxolitinib tablet twice a day.	
Subject analysis set title	≥ 6y - < 12y RUX 5mg BID (Tablet)
Subject analysis set type	Sub-group analysis
Subject analysis set description:	
Participants in this group were administered 5mg ruxolitinib tablets twice a day.	
Subject analysis set title	≥ 6y - < 12y RUX 5mg BID (Liquid)
Subject analysis set type	Sub-group analysis
Subject analysis set description:	
Participants in this group were administered ruxolitinib oral pediatric formulation twice a day.	
Subject analysis set title	≥ 2y - < 6y RUX 4mg/m2 BID (Liquid)
Subject analysis set type	Sub-group analysis
Subject analysis set description:	
Participants in this group were administered ruxolitinib oral pediatric formulation twice a day.	
Subject analysis set title	All Participants
Subject analysis set type	Sub-group analysis
Subject analysis set description:	
DOR was analyzed for all responders from the three age groups (Groups 1, 2 and 3)	
Subject analysis set title	All Participants
Subject analysis set type	Full analysis
Subject analysis set description:	
FFS was analyzed for all participants from the three age groups (Groups 1, 2 and 3)	
Subject analysis set title	All Participants
Subject analysis set type	Sub-group analysis
Subject analysis set description:	
MR was analyzed for all participants with underlying hematologic malignant disease from the three age groups (Groups 1, 2 and 3).	
Subject analysis set title	All Participants
Subject analysis set type	Full analysis
Subject analysis set description:	
NRM was analyzed for all participants from the three age groups (Groups 1, 2 and 3).	
Subject analysis set title	All Participants
Subject analysis set type	Full analysis

Subject analysis set description:

OS was analyzed for all participants from the three age groups (Groups 1, 2 and 3).

Subject analysis set title	All Participants
Subject analysis set type	Sub-group analysis

Subject analysis set description:

The percentage of participants with ≥50% reduction from baseline in daily corticosteroid dose at least once was analyzed for all participants in Groups 1, 2 and 3 who received corticosteroids at baseline.

Subject analysis set title	All Participants
Subject analysis set type	Sub-group analysis

Subject analysis set description:

The percentage of participants with reduction to <=0.2mg/kg/day from baseline in daily corticosteroid dose at least once was analyzed for all participants in Groups 1, 2 and 3 who received corticosteroids at baseline.

Subject analysis set title	All Participants
Subject analysis set type	Full analysis

Subject analysis set description:

Graft Failure was analyzed for all participants from the three age groups (Groups 1, 2 and 3).

Primary: Overall response rate (ORR) at Cycle 7 Day 1

End point title	Overall response rate (ORR) at Cycle 7 Day 1[1]

End point description:

ORR is defined as the percentage of participants demonstrating a complete response (CR) or partial response (PR) without the requirement of additional systemic therapies for an earlier progression, mixed response or non-response. The response is assessed per National Institute of Health (NIH) consensus criteria and scoring of response was relative to the organ stage at the start of study treatment. Full Analysis Set (FAS) comprised all subjects to whom study treatment has been assigned and who received at least one dose of study treatment.

End point type	Primary

End point timeframe:

At Cycle 7 Day 1 (Day 168); Cycle = 28 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: No statistical analysis was done

End point values	≥ 12y - < 18y RUX 10mg BID (Group 1)	≥ 6y - < 12y RUX 5mg BID (Group 2)	≥ 2y - < 6y RUX 4mg/m2 BID (Group 3)	All Participants
Subject group type	Reporting group	Reporting group	Reporting group	Subject analysis set
Number of subjects analysed	22	16	7	45
Units: Percentage of participants				
number (confidence interval 90%)	36.4 (19.6 to 56.1)	50.0 (27.9 to 72.1)	28.6 (5.3 to 65.9)	40.0 (27.7 to 53.3)

Statistical analyses

No statistical analyses for this end point

Secondary: Ruxolitinib concentrations by timepoint

End point title	Ruxolitinib concentrations by timepoint

End point description:

Pharmacokinetics (PK) of ruxolitinib by age groups (and formulation tablet vs liquid).

End point type	Secondary

End point timeframe:

Cycle 1 Day 1: 0.5, 2 and 6 hours post-dose; Pre-dose on Cycle 1 Day 8, Cycle 1 Day 15, Cycle 1 Day 22, Cycle 3 Day 1, Cycle 5 Day 1 and Cycle 7 Day 1; Cycle = 28 Days

End point values	≥ 12y - < 18y RUX 10mg BID (Tablet)	≥ 6y - < 12y RUX 5mg BID (Tablet)	≥ 6y - < 12y RUX 5mg BID (Liquid)	≥ 2y - < 6y RUX 4mg/m2 BID (Liquid)
Subject group type	Subject analysis set	Subject analysis set	Subject analysis set	Subject analysis set
Number of subjects analysed	20	13	2	7
Units: ug/ml				
geometric mean (geometric coefficient of variation)				
Cycle 1 Day 1: 0.5 hr (post-dose) (n =7,9,1,7)	60.0 (± 350.7)	32.4 (± 471.9)	78.6 (± 999)	60.1 (± 98.7)
Cycle 1 Day 1: 2 hr (post-dose) (n = 7,7,1,7)	112 (± 51.8)	106 (± 39.2)	77.0 (± 999)	49.7 (± 30.7)
Cycle 1 Day 1: 6hr (post-dose) (n = 7, 8, 1, 7)	56.7 (± 73.9)	47.8 (± 164.1)	60.0 (± 999)	15.3 (± 66.2)
Cycle 1 Day 8: 0 hr (pre-dose) (n =20, 13, 1, 7)	16.7 (± 208.3)	10.9 (± 146.5)	3.47 (± 999)	3.88 (± 152.4)
Cycle 1 Day 15: 0 hr (pre-dose) (n = 19, 12, 2, 7)	17.8 (± 195.5)	12.9 (± 248.2)	4.14 (± 399.3)	9.43 (± 121.5)
Cycle 1 Day 22: 0 hr (pre-dose) (n = 18, 11, 2, 7)	20.8 (± 199.5)	11.7 (± 165.4)	4.89 (± 505.2)	10.3 (± 150.4)
Cycle 3 Day 1: 0 hr (pre-dose (n = 17, 11, 2, 5)	11.4 (± 193.8)	11.3 (± 193.1)	2.66 (± 587.5)	5.56 (± 180.8)
Cycle 5 Day 1: 0 hr (pre-dose (n = 14,10, 2, 4)	13.0 (± 176.6)	16.4 (± 470.7)	11.0 (± 83.5)	7.32 (± 102.0)
Cycle 7 Day 1: 0 hr (pre-dose (n = 12, 8, 2, 4)	14.3 (± 173.7)	10.7 (± 129.9)	5.79 (± 120.1)	4.94 (± 165.9)

Statistical analyses

No statistical analyses for this end point

Secondary: Duration of response (DOR)

End point title	Duration of response (DOR)

End point description:

Time from first response until chronic Graft vs. host disease (cGvHD) progression, death, or the date of addition of systemic therapies for cGvHD assessed for responders only based on BOR up to Cycle 7 Day 1. Presented as percentage of participants to still be in response at different time points in months (per Kaplan-Meier estimates). Participants without event will be censored at the date of their last response assessment prior to or at the analysis cut-off date if no events occurred on or before 12 weeks (84 days) after the last GvHD assessment. As planned in the SAP, this outcome measure is provided for all participants instead of per age groups.
Full Analysis Set (FAS) comprised all subjects to whom study treatment has been assigned and who received at least one dose of study treatment.

End point type	Secondary

End point timeframe:

From baseline up to 39 cycles; Cycle = 28 Days

End point values	All Participants			
Subject group type	Subject analysis set			
Number of subjects analysed	37			
Units: Percentage of participants				
number (confidence interval 95%)				
Month 1	94.59 (80.07 to 98.62)			
Month 2	86.23 (70.01 to 94.03)			
Month 6	74.24 (56.17 to 85.74)			
Month 12	63.10 (43.74 to 77.38)			
Month 18	58.89 (39.23 to 74.12)			
Month 24	58.89 (39.23 to 74.12)			
Month 30	58.89 (39.23 to 74.12)			
Month 36	58.89 (39.23 to 74.12)			

Statistical analyses

No statistical analyses for this end point

Secondary: Overall Response Rate (ORR) at Cycle 4 Day 1

End point title	Overall Response Rate (ORR) at Cycle 4 Day 1

End point description:

ORR is defined as the percentage of participants demonstrating a complete response (CR) or partial response (PR) without the requirement of additional systemic therapies for an earlier progression, mixed response or non-response. The response is assessed per National Institute of Health (NIH) consensus criteria and scoring of response will be relative to the organ stage at the start of study treatment at Cycle 4 Day 1.

End point type	Secondary

End point timeframe:

At Cycle 4 Day 1 (Day 84); Cycle = 28 Days

End point values	≥ 12y - < 18y RUX 10mg BID (Group 1)	≥ 6y - < 12y RUX 5mg BID (Group 2)	≥ 2y - < 6y RUX 4mg/m2 BID (Group 3)	All Participants
Subject group type	Reporting group	Reporting group	Reporting group	Subject analysis set
Number of subjects analysed	22	16	7	45
Units: Percentage of participants				
number (confidence interval 90%)	54.5 (35.3 to 72.9)	62.5 (39.1 to 82.2)	42.9 (12.9 to 77.5)	55.6 (42.3 to 68.3)

Statistical analyses

No statistical analyses for this end point

Secondary: Best overall response (BOR)

End point title	Best overall response (BOR)

End point description:

Percentage of participants who achieved overall response (complete response (CR) or partial response (PR)) at any time point until Cycle 7 Day 1 or the start of additional systemic therapy for chronic GvHD.

End point type	Secondary

End point timeframe:

Until Cycle 7 Day 1 (Day 168) or the start of additional systemic therapy for cGvHD; Cycle = 28 Days

End point values	≥ 12y - < 18y RUX 10mg BID (Group 1)	≥ 6y - < 12y RUX 5mg BID (Group 2)	≥ 2y - < 6y RUX 4mg/m2 BID (Group 3)	All Participants
Subject group type	Reporting group	Reporting group	Reporting group	Subject analysis set
Number of subjects analysed	22	16	7	45
Units: Percentage of participants				
number (confidence interval 90%)	81.8 (63.1 to 93.5)	81.3 (58.3 to 94.7)	85.7 (47.9 to 99.3)	82.2 (70.2 to 90.8)

Statistical analyses

No statistical analyses for this end point

Secondary: Failure free survival (FFS)

End point title	Failure free survival (FFS)

End point description:

Failure-free survival was defined as the time from date of treatment to any of the following events: i) relapse or recurrence of underlying disease or death due to underlying disease, ii) non-relapse mortality, or iii) addition or initiation of another systemic therapy for cGvHD per Kaplan-Meier estimates. As planned in the SAP, this outcome measure is provided for all participants instead of per age groups.

End point type	Secondary

End point timeframe:

From baseline up to 39 cycles; Cycle = 28 Days

End point values	All Participants			
Subject group type	Subject analysis set			
Number of subjects analysed	45			
Units: Percentage of participants				
number (confidence interval 95%)				
Month 1	91.11 (78.03 to 96.57)			
Month 2	84.44 (70.12 to 92.26)			
Month 6	68.89 (53.20 to 80.25)			
Month 12	64.44 (48.67 to 76.48)			
Month 18	57.78 (42.11 to 70.61)			
Month 24	57.78 (42.11 to 70.61)			
Month 30	57.78 (42.11 to 70.61)			
Month 36	57.78 (42.11 to 70.61)			

Statistical analyses

No statistical analyses for this end point

Secondary: Cumulative incidence of malignancy relapse/recurrence (MR)

End point title	Cumulative incidence of malignancy relapse/recurrence (MR)

End point description:

MR was defined as the time from date of treatment assignment to the date of hematologic malignancy relapse/recurrence. Calculated for subjects with underlying hematologic malignant disease. The cumulative incidence (CI) of malignancy relapse/recurrence at 1, 2, 6, 12, 18, 24, 30 and 36 months after start of treatment has been reported. As planned in the SAP, this outcome measure is provided for all participants instead of per age groups.

End point type	Secondary

End point timeframe:

From baseline up to 39 cycles; Cycle = 28 Days

End point values	All Participants			
Subject group type	Subject analysis set			
Number of subjects analysed	30			
Units: Percentage of participants				
number (confidence interval 95%)				
Month 1	3.33 (0.23 to 14.80)			
Month 2	10.00 (2.47 to 23.88)			
Month 6	10.00 (2.47 to 23.88)			
Month 12	10.00 (2.47 to 23.88)			

Month 18	10.00 (2.47 to 23.88)			
Month 24	10.00 (2.47 to 23.88)			
Month 30	10.00 (2.47 to 23.88)			
Month 36	10.00 (2.47 to 23.88)			

Statistical analyses

No statistical analyses for this end point

Secondary: Non-relapse mortality (NRM)

End point title	Non-relapse mortality (NRM)

End point description:

NRM is defined as the time from date of treatment assignment to date of death not preceded by underlying disease relapse/recurrence calculated for all participants. The cumulative incidence (CI) of non-relapse mortality at 1, 2, 6, 12, 18, 24, 30 and 36 months after start of treatment has been reported. As planned in the SAP, this outcome measure is provided for all participants instead of per age groups.

End point type	Secondary

End point timeframe:

From baseline up to 39 cycles; Cycle = 28 Days

End point values	All Participants			
Subject group type	Subject analysis set			
Number of subjects analysed	45			
Units: Percentage of participants				
number (confidence interval 95%)				
Month 1	2.22 (0.17 to 10.28)			
Month 2	2.22 (0.17 to 10.28)			
Month 6	6.67 (1.70 to 16.53)			
Month 12	13.33 (5.34 to 25.02)			
Month 18	17.78 (8.23 to 30.28)			
Month 24	20.07 (9.80 to 32.93)			
Month 30	20.07 (9.80 to 32.93)			
Month 36	20.07 (9.80 to 32.93)			

Statistical analyses

No statistical analyses for this end point

Secondary: Overall survival (OS)

End point title	Overall survival (OS)

End point description:

OS is defined as the time from the date of treatment assignment to the date of death due to any cause. As planned in the SAP, this outcome measure is provided for all participants instead of per age groups.

End point type	Secondary

End point timeframe:

From baseline up to 39 cycles; Cycle = 28 Days

End point values	All Participants			
Subject group type	Subject analysis set			
Number of subjects analysed	45			
Units: survival probability in percentage				
number (confidence interval 95%)				
Month 1	97.78 (85.25 to 99.68)			
Month 2	97.78 (85.25 to 99.68)			
Month 6	91.06 (77.90 to 96.55)			
Month 12	84.23 (69.73 to 92.15)			
Month 18	79.67 (64.57 to 88.87)			
Month 24	77.33 (61.94 to 87.11)			
Month 30	77.33 (61.94 to 87.11)			
Month 36	74.91 (59.26 to 85.26)			

Statistical analyses

No statistical analyses for this end point

Secondary: Percentage of participants with ≥50% reduction from baseline in daily corticosteroid dose

End point title	Percentage of participants with ≥50% reduction from baseline in daily corticosteroid dose

End point description:

Reduction of at least ≥50% from baseline in daily corticosteroid use by Cycle 7 Day 1 (regardless of reason). As planned in the SAP, this outcome measure is provided for all participants instead of per age groups, for those who received corticosteroids at baseline.

End point type	Secondary

End point timeframe:

Baseline to Cycle 7 Day 1 (Day 168); Cycle = 28 Days

End point values	All Participants			
Subject group type	Subject analysis set			
Number of subjects analysed	40			
Units: Percentage of participants				
number (not applicable)	75.0			

Statistical analyses

No statistical analyses for this end point

Secondary: Graft failure

End point title	Graft failure

End point description:

Reported are the number of participants with graft failure from all age groups together. Graft failure was assessed by donor cell chimerism, defined as initial whole blood or marrow donor chimerism for those who had ≥ 5% donor cell chimerism at baseline. If donor cell chimerism declined to < 5% on subsequent measurements, graft failure was declared. As planned in the SAP, this outcome measure is provided for all participantsinstead of per age grpoups.

End point type	Secondary

End point timeframe:

From baseline up to 39 cycles; Cycle = 28 Days

End point values	All Participants			
Subject group type	Subject analysis set			
Number of subjects analysed	45			
Units: Participants	2			

Statistical analyses

No statistical analyses for this end point

Secondary: Percentage of participants with a reduction to a low dose corticosteriod

End point title	Percentage of participants with a reduction to a low dose corticosteriod

End point description:

Reduction to low dose corticosteroids, is defined as the percentage of participants with reduction from baseline in daily corticosteroid dose to methylprednisolone-equivalent steroid dose of ≤ 0.2 mg/kg/day (or equivalent dose of ≤ 0.25 mg/kg/day prednisone or prednisolone). As planned in the SAP, this outcome measure is provided for all participants instead of per age groups, for those who received corticosteroids at baseline.

End point type	Secondary

End point timeframe:
Baseline to Cycle 7 Day 1 (Day 168); Cycle = 28 Days

End point values	All Participants			
Subject group type	Subject analysis set			
Number of subjects analysed	40			
Units: Percentage of participants				
number (not applicable)	67.5			

Statistical analyses

No statistical analyses for this end point

Adverse events information

Timeframe for reporting adverse events:

AEs & on-treatment deaths were collected from 1st dose up to 30 days after last dose of study treatment.
Post-treatment survival follow-up events were collected 31 days after last dose of study treatment until study end, approx. 36 months/39 cycles.

Adverse event reporting additional description:

An Adverse Event (AE) is any sign or symptom that occurs during the conduct of the trial and post-treatment survival follow-up.

Assessment type	Systematic

Dictionary used

Dictionary name	MedDRA
Dictionary version	27.0

Reporting groups

Reporting group title	On-Treatment >=12y - <18y RUX 10mg BID

Reporting group description:

On-Treatment >=12y - <18y RUX 10mg BID

Reporting group title	On-Treatment >=6y - <12y RUX 5mg BID

Reporting group description:

On-Treatment >=6y - <12y RUX 5mg BID

Reporting group title	On-Treatment >=2y - <6y RUX 4mg/m2 BID

Reporting group description:

On-Treatment >=2y - <6y RUX 4mg/m2 BID

Reporting group title	On-Treatment All subjects

Reporting group description:

On-Treatment All subjects

Reporting group title	Post-Treatment >=12y - <18y RUX 10mg BID

Reporting group description:

Post-Treatment >=12y - <18y RUX 10mg BID

Reporting group title	Post-Treatment >=6y - <12y RUX 5mg BID

Reporting group description:

Post-Treatment >=6y - <12y RUX 5mg BID

Reporting group title	Post-Treatment >=2y - <6y RUX 4mg/m2 BID

Reporting group description:

Post-Treatment >=2y - <6y RUX 4mg/m2 BID

Reporting group title	Post-Treatment All subjects

Reporting group description:

Post-Treatment All subjects

Serious adverse events	On-Treatment >=12y - <18y RUX 10mg BID	On-Treatment >=6y - <12y RUX 5mg BID	On-Treatment >=2y - <6y RUX 4mg/m2 BID
Total subjects affected by serious adverse events			
subjects affected / exposed	15 / 22 (68.18%)	7 / 16 (43.75%)	4 / 7 (57.14%)
number of deaths (all causes)	0	2	1
number of deaths resulting from adverse events	0	0	0

Neoplasms benign, malignant and unspecified (incl cysts and polyps)			
Post transplant lymphoproliferative disorder			
subjects affected / exposed	1 / 22 (4.55%)	0 / 16 (0.00%)	0 / 7 (0.00%)
occurrences causally related to treatment / all	0 / 1	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
General disorders and administration site conditions			
Pyrexia			
subjects affected / exposed	2 / 22 (9.09%)	0 / 16 (0.00%)	1 / 7 (14.29%)
occurrences causally related to treatment / all	0 / 3	0 / 0	0 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Oedema peripheral			
subjects affected / exposed	1 / 22 (4.55%)	0 / 16 (0.00%)	0 / 7 (0.00%)
occurrences causally related to treatment / all	1 / 1	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Immune system disorders			
Haemophagocytic lymphohistiocytosis			
subjects affected / exposed	1 / 22 (4.55%)	0 / 16 (0.00%)	0 / 7 (0.00%)
occurrences causally related to treatment / all	0 / 1	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Respiratory, thoracic and mediastinal disorders			
Acute respiratory distress syndrome			
subjects affected / exposed	0 / 22 (0.00%)	1 / 16 (6.25%)	0 / 7 (0.00%)
occurrences causally related to treatment / all	0 / 0	0 / 1	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 1	0 / 0
Alveolar proteinosis			
subjects affected / exposed	1 / 22 (4.55%)	0 / 16 (0.00%)	0 / 7 (0.00%)
occurrences causally related to treatment / all	1 / 1	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Dyspnoea			
subjects affected / exposed	1 / 22 (4.55%)	0 / 16 (0.00%)	0 / 7 (0.00%)
occurrences causally related to treatment / all	0 / 1	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Pneumothorax			

subjects affected / exposed	0 / 22 (0.00%)	1 / 16 (6.25%)	0 / 7 (0.00%)
occurrences causally related to treatment / all	0 / 0	0 / 1	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Respiratory distress			
subjects affected / exposed	0 / 22 (0.00%)	1 / 16 (6.25%)	0 / 7 (0.00%)
occurrences causally related to treatment / all	0 / 0	0 / 1	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Investigations			
Blood creatinine increased			
subjects affected / exposed	0 / 22 (0.00%)	1 / 16 (6.25%)	0 / 7 (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
Injury, poisoning and procedural complications			
Transfusion-related acute lung injury			
subjects affected / exposed	0 / 22 (0.00%)	1 / 16 (6.25%)	0 / 7 (0.00%)
occurrences causally related to treatment / all	0 / 0	0 / 1	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Transplant failure			
subjects affected / exposed	1 / 22 (4.55%)	0 / 16 (0.00%)	0 / 7 (0.00%)
occurrences causally related to treatment / all	0 / 1	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Cardiac disorders			
Bradycardia			
subjects affected / exposed	0 / 22 (0.00%)	0 / 16 (0.00%)	0 / 7 (0.00%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Nervous system disorders			
Aura			
subjects affected / exposed	1 / 22 (4.55%)	0 / 16 (0.00%)	0 / 7 (0.00%)
occurrences causally related to treatment / all	0 / 1	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Presyncope			

subjects affected / exposed	1 / 22 (4.55%)	0 / 16 (0.00%)	0 / 7 (0.00%)
occurrences causally related to treatment / all	0 / 1	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Blood and lymphatic system disorders			
Febrile neutropenia			
subjects affected / exposed	0 / 22 (0.00%)	0 / 16 (0.00%)	1 / 7 (14.29%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Thrombocytopenia			
subjects affected / exposed	1 / 22 (4.55%)	0 / 16 (0.00%)	0 / 7 (0.00%)
occurrences causally related to treatment / all	1 / 1	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Eye disorders			
Retinal vein occlusion			
subjects affected / exposed	0 / 22 (0.00%)	1 / 16 (6.25%)	0 / 7 (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
Gastrointestinal disorders			
Pneumatosis intestinalis			
subjects affected / exposed	1 / 22 (4.55%)	0 / 16 (0.00%)	0 / 7 (0.00%)
occurrences causally related to treatment / all	0 / 1	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Hepatobiliary disorders			
Hepatorenal syndrome			
subjects affected / exposed	0 / 22 (0.00%)	1 / 16 (6.25%)	0 / 7 (0.00%)
occurrences causally related to treatment / all	0 / 0	0 / 1	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Renal and urinary disorders			
Urinary retention			
subjects affected / exposed	1 / 22 (4.55%)	0 / 16 (0.00%)	0 / 7 (0.00%)
occurrences causally related to treatment / all	0 / 1	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Musculoskeletal and connective tissue disorders			
Muscular weakness			

subjects affected / exposed	2 / 22 (9.09%)	0 / 16 (0.00%)	0 / 7 (0.00%)
occurrences causally related to treatment / all	0 / 2	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Pain in extremity			
subjects affected / exposed	1 / 22 (4.55%)	0 / 16 (0.00%)	0 / 7 (0.00%)
occurrences causally related to treatment / all	1 / 1	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Infections and infestations			
Aspergillus infection			
subjects affected / exposed	0 / 22 (0.00%)	0 / 16 (0.00%)	1 / 7 (14.29%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 1
COVID-19			
subjects affected / exposed	2 / 22 (9.09%)	0 / 16 (0.00%)	0 / 7 (0.00%)
occurrences causally related to treatment / all	0 / 2	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Cytomegalovirus infection reactivation			
subjects affected / exposed	1 / 22 (4.55%)	0 / 16 (0.00%)	0 / 7 (0.00%)
occurrences causally related to treatment / all	1 / 1	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Epstein-Barr virus infection			
subjects affected / exposed	1 / 22 (4.55%)	0 / 16 (0.00%)	0 / 7 (0.00%)
occurrences causally related to treatment / all	0 / 1	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Dengue fever			
subjects affected / exposed	1 / 22 (4.55%)	0 / 16 (0.00%)	0 / 7 (0.00%)
occurrences causally related to treatment / all	0 / 1	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Herpes zoster			
subjects affected / exposed	2 / 22 (9.09%)	0 / 16 (0.00%)	0 / 7 (0.00%)
occurrences causally related to treatment / all	1 / 2	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Gastroenteritis viral			

subjects affected / exposed	1 / 22 (4.55%)	0 / 16 (0.00%)	0 / 7 (0.00%)
occurrences causally related to treatment / all	0 / 1	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Human bocavirus infection			
subjects affected / exposed	0 / 22 (0.00%)	0 / 16 (0.00%)	1 / 7 (14.29%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Infectious pleural effusion			
subjects affected / exposed	0 / 22 (0.00%)	1 / 16 (6.25%)	0 / 7 (0.00%)
occurrences causally related to treatment / all	0 / 0	0 / 1	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Parainfluenzae virus infection			
subjects affected / exposed	0 / 22 (0.00%)	0 / 16 (0.00%)	1 / 7 (14.29%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Pneumonia			
subjects affected / exposed	1 / 22 (4.55%)	1 / 16 (6.25%)	0 / 7 (0.00%)
occurrences causally related to treatment / all	0 / 1	0 / 2	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Pneumonia fungal			
subjects affected / exposed	1 / 22 (4.55%)	0 / 16 (0.00%)	0 / 7 (0.00%)
occurrences causally related to treatment / all	1 / 1	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Pneumonia respiratory syncytial viral			
subjects affected / exposed	1 / 22 (4.55%)	0 / 16 (0.00%)	0 / 7 (0.00%)
occurrences causally related to treatment / all	0 / 1	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Pneumonia viral			
subjects affected / exposed	0 / 22 (0.00%)	0 / 16 (0.00%)	1 / 7 (14.29%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 2
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Rhinovirus infection			

subjects affected / exposed	0 / 22 (0.00%)	0 / 16 (0.00%)	1 / 7 (14.29%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Respiratory syncytial virus infection			
subjects affected / exposed	0 / 22 (0.00%)	1 / 16 (6.25%)	0 / 7 (0.00%)
occurrences causally related to treatment / all	0 / 0	0 / 1	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Septic shock			
subjects affected / exposed	0 / 22 (0.00%)	1 / 16 (6.25%)	0 / 7 (0.00%)
occurrences causally related to treatment / all	0 / 0	0 / 1	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 1	0 / 0
Sepsis			
subjects affected / exposed	1 / 22 (4.55%)	0 / 16 (0.00%)	0 / 7 (0.00%)
occurrences causally related to treatment / all	0 / 1	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Tooth infection			
subjects affected / exposed	0 / 22 (0.00%)	0 / 16 (0.00%)	1 / 7 (14.29%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Upper respiratory tract infection			
subjects affected / exposed	0 / 22 (0.00%)	0 / 16 (0.00%)	1 / 7 (14.29%)
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 infection			
subjects affected / exposed	0 / 22 (0.00%)	1 / 16 (6.25%)	0 / 7 (0.00%)
occurrences causally related to treatment / all	0 / 0	0 / 1	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Metabolism and nutrition disorders			
Steroid diabetes			
subjects affected / exposed	1 / 22 (4.55%)	0 / 16 (0.00%)	0 / 7 (0.00%)
occurrences causally related to treatment / all	0 / 1	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Hyponatraemia			

subjects affected / exposed	2 / 22 (9.09%)	0 / 16 (0.00%)	0 / 7 (0.00%)
occurrences causally related to treatment / all	2 / 2	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0

Serious adverse events	On-Treatment All subjects	Post-Treatment >=12y - <18y RUX 10mg BID	Post-Treatment >=6y - <12y RUX 5mg BID
Total subjects affected by serious adverse events			
subjects affected / exposed	26 / 45 (57.78%)	3 / 22 (13.64%)	0 / 16 (0.00%)
number of deaths (all causes)	3	6	1
number of deaths resulting from adverse events	0	1	0
Neoplasms benign, malignant and unspecified (incl cysts and polyps)			
Post transplant lymphoproliferative disorder			
subjects affected / exposed	1 / 45 (2.22%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences causally related to treatment / all	0 / 1	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
General disorders and administration site conditions			
Pyrexia			
subjects affected / exposed	3 / 45 (6.67%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences causally related to treatment / all	0 / 4	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Oedema peripheral			
subjects affected / exposed	1 / 45 (2.22%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences causally related to treatment / all	1 / 1	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Immune system disorders			
Haemophagocytic lymphohistiocytosis			
subjects affected / exposed	1 / 45 (2.22%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences causally related to treatment / all	0 / 1	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Respiratory, thoracic and mediastinal disorders			
Acute respiratory distress syndrome			
subjects affected / exposed	1 / 45 (2.22%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences causally related to treatment / all	0 / 1	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 1	0 / 0	0 / 0

Alveolar proteinosis			
subjects affected / exposed	1 / 45 (2.22%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences causally related to treatment / all	1 / 1	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Dyspnoea			
subjects affected / exposed	1 / 45 (2.22%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences causally related to treatment / all	0 / 1	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Pneumothorax			
subjects affected / exposed	1 / 45 (2.22%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences causally related to treatment / all	0 / 1	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Respiratory distress			
subjects affected / exposed	1 / 45 (2.22%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences causally related to treatment / all	0 / 1	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Investigations			
Blood creatinine increased			
subjects affected / exposed	1 / 45 (2.22%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences causally related to treatment / all	1 / 1	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Injury, poisoning and procedural complications			
Transfusion-related acute lung injury			
subjects affected / exposed	1 / 45 (2.22%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences causally related to treatment / all	0 / 1	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Transplant failure			
subjects affected / exposed	1 / 45 (2.22%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences causally related to treatment / all	0 / 1	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Cardiac disorders			
Bradycardia			

subjects affected / exposed	0 / 45 (0.00%)	1 / 22 (4.55%)	0 / 16 (0.00%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Nervous system disorders			
Aura			
subjects affected / exposed	1 / 45 (2.22%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences causally related to treatment / all	0 / 1	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Presyncope			
subjects affected / exposed	1 / 45 (2.22%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences causally related to treatment / all	0 / 1	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Blood and lymphatic system disorders			
Febrile neutropenia			
subjects affected / exposed	1 / 45 (2.22%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences causally related to treatment / all	0 / 1	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Thrombocytopenia			
subjects affected / exposed	1 / 45 (2.22%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences causally related to treatment / all	1 / 1	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Eye disorders			
Retinal vein occlusion			
subjects affected / exposed	1 / 45 (2.22%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences causally related to treatment / all	1 / 1	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Gastrointestinal disorders			
Pneumatosis intestinalis			
subjects affected / exposed	1 / 45 (2.22%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences causally related to treatment / all	0 / 1	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Hepatobiliary disorders			
Hepatorenal syndrome			

subjects affected / exposed	1 / 45 (2.22%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences causally related to treatment / all	0 / 1	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Renal and urinary disorders			
Urinary retention			
subjects affected / exposed	1 / 45 (2.22%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences causally related to treatment / all	0 / 1	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Musculoskeletal and connective tissue disorders			
Muscular weakness			
subjects affected / exposed	2 / 45 (4.44%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences causally related to treatment / all	0 / 2	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Pain in extremity			
subjects affected / exposed	1 / 45 (2.22%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences causally related to treatment / all	1 / 1	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Infections and infestations			
Aspergillus infection			
subjects affected / exposed	1 / 45 (2.22%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences causally related to treatment / all	0 / 1	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 1	0 / 0	0 / 0
COVID-19			
subjects affected / exposed	2 / 45 (4.44%)	1 / 22 (4.55%)	0 / 16 (0.00%)
occurrences causally related to treatment / all	0 / 2	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 1	0 / 0
Cytomegalovirus infection reactivation			
subjects affected / exposed	1 / 45 (2.22%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences causally related to treatment / all	1 / 1	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Epstein-Barr virus infection			

subjects affected / exposed	1 / 45 (2.22%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences causally related to treatment / all	0 / 1	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Dengue fever			
subjects affected / exposed	1 / 45 (2.22%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences causally related to treatment / all	0 / 1	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Herpes zoster			
subjects affected / exposed	2 / 45 (4.44%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences causally related to treatment / all	1 / 2	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Gastroenteritis viral			
subjects affected / exposed	1 / 45 (2.22%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences causally related to treatment / all	0 / 1	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Human bocavirus infection			
subjects affected / exposed	1 / 45 (2.22%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences causally related to treatment / all	0 / 1	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Infectious pleural effusion			
subjects affected / exposed	1 / 45 (2.22%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences causally related to treatment / all	0 / 1	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Parainfluenzae virus infection			
subjects affected / exposed	1 / 45 (2.22%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences causally related to treatment / all	0 / 1	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Pneumonia			
subjects affected / exposed	2 / 45 (4.44%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences causally related to treatment / all	0 / 3	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Pneumonia fungal			

subjects affected / exposed	1 / 45 (2.22%)	1 / 22 (4.55%)	0 / 16 (0.00%)
occurrences causally related to treatment / all	1 / 1	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	1 / 1	0 / 0
Pneumonia respiratory syncytial viral			
subjects affected / exposed	1 / 45 (2.22%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences causally related to treatment / all	0 / 1	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Pneumonia viral			
subjects affected / exposed	1 / 45 (2.22%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences causally related to treatment / all	0 / 2	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Rhinovirus infection			
subjects affected / exposed	1 / 45 (2.22%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences causally related to treatment / all	0 / 1	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Respiratory syncytial virus infection			
subjects affected / exposed	1 / 45 (2.22%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences causally related to treatment / all	0 / 1	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Septic shock			
subjects affected / exposed	1 / 45 (2.22%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences causally related to treatment / all	0 / 1	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 1	0 / 0	0 / 0
Sepsis			
subjects affected / exposed	1 / 45 (2.22%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences causally related to treatment / all	0 / 1	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Tooth infection			
subjects affected / exposed	1 / 45 (2.22%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences causally related to treatment / all	0 / 1	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Upper respiratory tract infection			

subjects affected / exposed	1 / 45 (2.22%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences causally related to treatment / all	0 / 1	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Urinary tract infection			
subjects affected / exposed	1 / 45 (2.22%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences causally related to treatment / all	0 / 1	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Metabolism and nutrition disorders			
Steroid diabetes			
subjects affected / exposed	1 / 45 (2.22%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences causally related to treatment / all	0 / 1	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Hyponatraemia			
subjects affected / exposed	2 / 45 (4.44%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences causally related to treatment / all	2 / 2	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0

Serious adverse events	Post-Treatment >=2y - <6y RUX 4mg/m2 BID	Post-Treatment All subjects	
Total subjects affected by serious adverse events			
subjects affected / exposed	0 / 7 (0.00%)	3 / 45 (6.67%)	
number of deaths (all causes)	1	8	
number of deaths resulting from adverse events	0	1	
Neoplasms benign, malignant and unspecified (incl cysts and polyps)			
Post transplant lymphoproliferative disorder			
subjects affected / exposed	0 / 7 (0.00%)	0 / 45 (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			
Pyrexia			
subjects affected / exposed	0 / 7 (0.00%)	0 / 45 (0.00%)	
occurrences causally related to treatment / all	0 / 0	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Oedema peripheral			

subjects affected / exposed	0 / 7 (0.00%)	0 / 45 (0.00%)	
occurrences causally related to treatment / all	0 / 0	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Immune system disorders			
Haemophagocytic lymphohistiocytosis			
subjects affected / exposed	0 / 7 (0.00%)	0 / 45 (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 distress syndrome			
subjects affected / exposed	0 / 7 (0.00%)	0 / 45 (0.00%)	
occurrences causally related to treatment / all	0 / 0	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Alveolar proteinosis			
subjects affected / exposed	0 / 7 (0.00%)	0 / 45 (0.00%)	
occurrences causally related to treatment / all	0 / 0	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Dyspnoea			
subjects affected / exposed	0 / 7 (0.00%)	0 / 45 (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 / 7 (0.00%)	0 / 45 (0.00%)	
occurrences causally related to treatment / all	0 / 0	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Respiratory distress			
subjects affected / exposed	0 / 7 (0.00%)	0 / 45 (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 / 7 (0.00%)	0 / 45 (0.00%)	
occurrences causally related to treatment / all	0 / 0	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Injury, poisoning and procedural complications			
Transfusion-related acute lung injury			
subjects affected / exposed	0 / 7 (0.00%)	0 / 45 (0.00%)	
occurrences causally related to treatment / all	0 / 0	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Transplant failure			
subjects affected / exposed	0 / 7 (0.00%)	0 / 45 (0.00%)	
occurrences causally related to treatment / all	0 / 0	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Cardiac disorders			
Bradycardia			
subjects affected / exposed	0 / 7 (0.00%)	1 / 45 (2.22%)	
occurrences causally related to treatment / all	0 / 0	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Nervous system disorders			
Aura			
subjects affected / exposed	0 / 7 (0.00%)	0 / 45 (0.00%)	
occurrences causally related to treatment / all	0 / 0	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Presyncope			
subjects affected / exposed	0 / 7 (0.00%)	0 / 45 (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 / 7 (0.00%)	0 / 45 (0.00%)	
occurrences causally related to treatment / all	0 / 0	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Thrombocytopenia			

subjects affected / exposed	0 / 7 (0.00%)	0 / 45 (0.00%)	
occurrences causally related to treatment / all	0 / 0	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Eye disorders			
Retinal vein occlusion			
subjects affected / exposed	0 / 7 (0.00%)	0 / 45 (0.00%)	
occurrences causally related to treatment / all	0 / 0	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Gastrointestinal disorders			
Pneumatosis intestinalis			
subjects affected / exposed	0 / 7 (0.00%)	0 / 45 (0.00%)	
occurrences causally related to treatment / all	0 / 0	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Hepatobiliary disorders			
Hepatorenal syndrome			
subjects affected / exposed	0 / 7 (0.00%)	0 / 45 (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			
Urinary retention			
subjects affected / exposed	0 / 7 (0.00%)	0 / 45 (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			
Muscular weakness			
subjects affected / exposed	0 / 7 (0.00%)	0 / 45 (0.00%)	
occurrences causally related to treatment / all	0 / 0	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Pain in extremity			
subjects affected / exposed	0 / 7 (0.00%)	0 / 45 (0.00%)	
occurrences causally related to treatment / all	0 / 0	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Infections and infestations			
Aspergillus infection			

subjects affected / exposed	0 / 7 (0.00%)	0 / 45 (0.00%)	
occurrences causally related to treatment / all	0 / 0	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
COVID-19			
subjects affected / exposed	0 / 7 (0.00%)	1 / 45 (2.22%)	
occurrences causally related to treatment / all	0 / 0	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 1	
Cytomegalovirus infection reactivation			
subjects affected / exposed	0 / 7 (0.00%)	0 / 45 (0.00%)	
occurrences causally related to treatment / all	0 / 0	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Epstein-Barr virus infection			
subjects affected / exposed	0 / 7 (0.00%)	0 / 45 (0.00%)	
occurrences causally related to treatment / all	0 / 0	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Dengue fever			
subjects affected / exposed	0 / 7 (0.00%)	0 / 45 (0.00%)	
occurrences causally related to treatment / all	0 / 0	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Herpes zoster			
subjects affected / exposed	0 / 7 (0.00%)	0 / 45 (0.00%)	
occurrences causally related to treatment / all	0 / 0	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Gastroenteritis viral			
subjects affected / exposed	0 / 7 (0.00%)	0 / 45 (0.00%)	
occurrences causally related to treatment / all	0 / 0	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Human bocavirus infection			
subjects affected / exposed	0 / 7 (0.00%)	0 / 45 (0.00%)	
occurrences causally related to treatment / all	0 / 0	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Infectious pleural effusion			

subjects affected / exposed	0 / 7 (0.00%)	0 / 45 (0.00%)	
occurrences causally related to treatment / all	0 / 0	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Parainfluenzae virus infection			
subjects affected / exposed	0 / 7 (0.00%)	0 / 45 (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 / 7 (0.00%)	0 / 45 (0.00%)	
occurrences causally related to treatment / all	0 / 0	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Pneumonia fungal			
subjects affected / exposed	0 / 7 (0.00%)	1 / 45 (2.22%)	
occurrences causally related to treatment / all	0 / 0	0 / 0	
deaths causally related to treatment / all	0 / 0	1 / 1	
Pneumonia respiratory syncytial viral			
subjects affected / exposed	0 / 7 (0.00%)	0 / 45 (0.00%)	
occurrences causally related to treatment / all	0 / 0	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Pneumonia viral			
subjects affected / exposed	0 / 7 (0.00%)	0 / 45 (0.00%)	
occurrences causally related to treatment / all	0 / 0	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Rhinovirus infection			
subjects affected / exposed	0 / 7 (0.00%)	0 / 45 (0.00%)	
occurrences causally related to treatment / all	0 / 0	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Respiratory syncytial virus infection			
subjects affected / exposed	0 / 7 (0.00%)	0 / 45 (0.00%)	
occurrences causally related to treatment / all	0 / 0	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Septic shock			

subjects affected / exposed	0 / 7 (0.00%)	0 / 45 (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 / 7 (0.00%)	0 / 45 (0.00%)	
occurrences causally related to treatment / all	0 / 0	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Tooth infection			
subjects affected / exposed	0 / 7 (0.00%)	0 / 45 (0.00%)	
occurrences causally related to treatment / all	0 / 0	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Upper respiratory tract infection			
subjects affected / exposed	0 / 7 (0.00%)	0 / 45 (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 / 7 (0.00%)	0 / 45 (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			
Steroid diabetes			
subjects affected / exposed	0 / 7 (0.00%)	0 / 45 (0.00%)	
occurrences causally related to treatment / all	0 / 0	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Hyponatraemia			
subjects affected / exposed	0 / 7 (0.00%)	0 / 45 (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	On-Treatment >=12y - <18y RUX 10mg BID	On-Treatment >=6y - <12y RUX 5mg BID	On-Treatment >=2y - <6y RUX 4mg/m2 BID
Total subjects affected by non-serious adverse events			
subjects affected / exposed	22 / 22 (100.00%)	15 / 16 (93.75%)	7 / 7 (100.00%)
Neoplasms benign, malignant and unspecified (incl cysts and polyps)			
Skin papilloma			
subjects affected / exposed	1 / 22 (4.55%)	1 / 16 (6.25%)	0 / 7 (0.00%)
occurrences (all)	1	1	0
Vascular disorders			
Hypertension			
subjects affected / exposed	3 / 22 (13.64%)	3 / 16 (18.75%)	1 / 7 (14.29%)
occurrences (all)	3	3	1
Hot flush			
subjects affected / exposed	0 / 22 (0.00%)	0 / 16 (0.00%)	1 / 7 (14.29%)
occurrences (all)	0	0	1
General disorders and administration site conditions			
Pyrexia			
subjects affected / exposed	3 / 22 (13.64%)	4 / 16 (25.00%)	0 / 7 (0.00%)
occurrences (all)	4	6	0
Pain			
subjects affected / exposed	3 / 22 (13.64%)	0 / 16 (0.00%)	0 / 7 (0.00%)
occurrences (all)	3	0	0
Oedema peripheral			
subjects affected / exposed	2 / 22 (9.09%)	0 / 16 (0.00%)	0 / 7 (0.00%)
occurrences (all)	3	0	0
Influenza like illness			
subjects affected / exposed	0 / 22 (0.00%)	1 / 16 (6.25%)	0 / 7 (0.00%)
occurrences (all)	0	1	0
Immune system disorders			
Allergy to chemicals			
subjects affected / exposed	0 / 22 (0.00%)	1 / 16 (6.25%)	0 / 7 (0.00%)
occurrences (all)	0	1	0
Reproductive system and breast disorders			
Pruritus genital			
subjects affected / exposed	0 / 22 (0.00%)	1 / 16 (6.25%)	0 / 7 (0.00%)
occurrences (all)	0	1	0
Acquired phimosis			

subjects affected / exposed	0 / 22 (0.00%)	1 / 16 (6.25%)	0 / 7 (0.00%)
occurrences (all)	0	1	0
Respiratory, thoracic and mediastinal disorders			
Acute respiratory failure			
subjects affected / exposed	0 / 22 (0.00%)	1 / 16 (6.25%)	0 / 7 (0.00%)
occurrences (all)	0	1	0
Cough			
subjects affected / exposed	3 / 22 (13.64%)	3 / 16 (18.75%)	0 / 7 (0.00%)
occurrences (all)	3	7	0
Epistaxis			
subjects affected / exposed	0 / 22 (0.00%)	1 / 16 (6.25%)	0 / 7 (0.00%)
occurrences (all)	0	1	0
Pneumothorax			
subjects affected / exposed	0 / 22 (0.00%)	1 / 16 (6.25%)	0 / 7 (0.00%)
occurrences (all)	0	2	0
Oropharyngeal pain			
subjects affected / exposed	2 / 22 (9.09%)	0 / 16 (0.00%)	0 / 7 (0.00%)
occurrences (all)	2	0	0
Productive cough			
subjects affected / exposed	1 / 22 (4.55%)	1 / 16 (6.25%)	0 / 7 (0.00%)
occurrences (all)	1	1	0
Rhinorrhoea			
subjects affected / exposed	0 / 22 (0.00%)	1 / 16 (6.25%)	0 / 7 (0.00%)
occurrences (all)	0	1	0
Psychiatric disorders			
Insomnia			
subjects affected / exposed	2 / 22 (9.09%)	1 / 16 (6.25%)	0 / 7 (0.00%)
occurrences (all)	2	1	0
Investigations			
Alanine aminotransferase increased			
subjects affected / exposed	3 / 22 (13.64%)	1 / 16 (6.25%)	1 / 7 (14.29%)
occurrences (all)	6	1	1
Amylase increased			
subjects affected / exposed	1 / 22 (4.55%)	1 / 16 (6.25%)	0 / 7 (0.00%)
occurrences (all)	1	1	0
Aspartate aminotransferase increased			

subjects affected / exposed	1 / 22 (4.55%)	1 / 16 (6.25%)	1 / 7 (14.29%)
occurrences (all)	3	1	1
Blood creatine phosphokinase increased			
subjects affected / exposed	0 / 22 (0.00%)	2 / 16 (12.50%)	0 / 7 (0.00%)
occurrences (all)	0	2	0
Blood cholesterol increased			
subjects affected / exposed	2 / 22 (9.09%)	0 / 16 (0.00%)	0 / 7 (0.00%)
occurrences (all)	2	0	0
Blood alkaline phosphatase increased			
subjects affected / exposed	0 / 22 (0.00%)	1 / 16 (6.25%)	1 / 7 (14.29%)
occurrences (all)	0	1	1
Blood lactate dehydrogenase increased			
subjects affected / exposed	0 / 22 (0.00%)	1 / 16 (6.25%)	0 / 7 (0.00%)
occurrences (all)	0	1	0
Gamma-glutamyltransferase increased			
subjects affected / exposed	2 / 22 (9.09%)	1 / 16 (6.25%)	0 / 7 (0.00%)
occurrences (all)	3	1	0
Epstein-Barr virus test positive			
subjects affected / exposed	0 / 22 (0.00%)	1 / 16 (6.25%)	0 / 7 (0.00%)
occurrences (all)	0	1	0
C-reactive protein increased			
subjects affected / exposed	0 / 22 (0.00%)	1 / 16 (6.25%)	1 / 7 (14.29%)
occurrences (all)	0	1	1
Hepatic enzyme increased			
subjects affected / exposed	0 / 22 (0.00%)	1 / 16 (6.25%)	0 / 7 (0.00%)
occurrences (all)	0	1	0
Blood magnesium increased			
subjects affected / exposed	0 / 22 (0.00%)	1 / 16 (6.25%)	0 / 7 (0.00%)
occurrences (all)	0	1	0
Neutrophil count decreased			
subjects affected / exposed	5 / 22 (22.73%)	2 / 16 (12.50%)	2 / 7 (28.57%)
occurrences (all)	9	3	2
Lymphocyte count decreased			

subjects affected / exposed	2 / 22 (9.09%)	0 / 16 (0.00%)	1 / 7 (14.29%)
occurrences (all)	3	0	2
Immunoglobulins decreased			
subjects affected / exposed	0 / 22 (0.00%)	1 / 16 (6.25%)	0 / 7 (0.00%)
occurrences (all)	0	1	0
Herpes simplex test positive			
subjects affected / exposed	0 / 22 (0.00%)	0 / 16 (0.00%)	1 / 7 (14.29%)
occurrences (all)	0	0	1
Platelet count decreased			
subjects affected / exposed	5 / 22 (22.73%)	2 / 16 (12.50%)	0 / 7 (0.00%)
occurrences (all)	6	2	0
Serum ferritin increased			
subjects affected / exposed	1 / 22 (4.55%)	1 / 16 (6.25%)	0 / 7 (0.00%)
occurrences (all)	1	1	0
Vascular access device culture positive			
subjects affected / exposed	0 / 22 (0.00%)	0 / 16 (0.00%)	1 / 7 (14.29%)
occurrences (all)	0	0	2
Weight decreased			
subjects affected / exposed	0 / 22 (0.00%)	2 / 16 (12.50%)	0 / 7 (0.00%)
occurrences (all)	0	2	0
Weight increased			
subjects affected / exposed	2 / 22 (9.09%)	0 / 16 (0.00%)	0 / 7 (0.00%)
occurrences (all)	2	0	0
White blood cell count decreased			
subjects affected / exposed	2 / 22 (9.09%)	2 / 16 (12.50%)	1 / 7 (14.29%)
occurrences (all)	4	2	1
Injury, poisoning and procedural complications			
Head injury			
subjects affected / exposed	0 / 22 (0.00%)	0 / 16 (0.00%)	1 / 7 (14.29%)
occurrences (all)	0	0	1
Fall			
subjects affected / exposed	0 / 22 (0.00%)	0 / 16 (0.00%)	1 / 7 (14.29%)
occurrences (all)	0	0	1
Extraskeletal ossification			

subjects affected / exposed	0 / 22 (0.00%)	1 / 16 (6.25%)	0 / 7 (0.00%)
occurrences (all)	0	1	0
Arthropod bite			
subjects affected / exposed	0 / 22 (0.00%)	1 / 16 (6.25%)	0 / 7 (0.00%)
occurrences (all)	0	1	0
Ligament sprain			
subjects affected / exposed	0 / 22 (0.00%)	1 / 16 (6.25%)	0 / 7 (0.00%)
occurrences (all)	0	1	0
Post procedural haemorrhage			
subjects affected / exposed	0 / 22 (0.00%)	1 / 16 (6.25%)	0 / 7 (0.00%)
occurrences (all)	0	1	0
Procedural pain			
subjects affected / exposed	0 / 22 (0.00%)	1 / 16 (6.25%)	0 / 7 (0.00%)
occurrences (all)	0	1	0
Ulna fracture			
subjects affected / exposed	0 / 22 (0.00%)	1 / 16 (6.25%)	0 / 7 (0.00%)
occurrences (all)	0	1	0
Cardiac disorders			
Tachycardia			
subjects affected / exposed	3 / 22 (13.64%)	1 / 16 (6.25%)	0 / 7 (0.00%)
occurrences (all)	4	1	0
Nervous system disorders			
Polyneuropathy			
subjects affected / exposed	0 / 22 (0.00%)	1 / 16 (6.25%)	0 / 7 (0.00%)
occurrences (all)	0	1	0
Headache			
subjects affected / exposed	5 / 22 (22.73%)	2 / 16 (12.50%)	0 / 7 (0.00%)
occurrences (all)	15	2	0
Blood and lymphatic system disorders			
Neutropenia			
subjects affected / exposed	2 / 22 (9.09%)	4 / 16 (25.00%)	2 / 7 (28.57%)
occurrences (all)	2	6	2
Lymphopenia			
subjects affected / exposed	0 / 22 (0.00%)	1 / 16 (6.25%)	0 / 7 (0.00%)
occurrences (all)	0	2	0
Anaemia			

subjects affected / exposed	7 / 22 (31.82%)	2 / 16 (12.50%)	2 / 7 (28.57%)
occurrences (all)	7	3	3
Coagulopathy			
subjects affected / exposed	0 / 22 (0.00%)	1 / 16 (6.25%)	0 / 7 (0.00%)
occurrences (all)	0	1	0
Leukopenia			
subjects affected / exposed	2 / 22 (9.09%)	1 / 16 (6.25%)	0 / 7 (0.00%)
occurrences (all)	3	1	0
Thrombocytopenia			
subjects affected / exposed	3 / 22 (13.64%)	1 / 16 (6.25%)	1 / 7 (14.29%)
occurrences (all)	4	1	1
Ear and labyrinth disorders			
Ear pain			
subjects affected / exposed	0 / 22 (0.00%)	1 / 16 (6.25%)	0 / 7 (0.00%)
occurrences (all)	0	1	0
Ear canal stenosis			
subjects affected / exposed	0 / 22 (0.00%)	1 / 16 (6.25%)	0 / 7 (0.00%)
occurrences (all)	0	1	0
Eye disorders			
Astigmatism			
subjects affected / exposed	0 / 22 (0.00%)	0 / 16 (0.00%)	1 / 7 (14.29%)
occurrences (all)	0	0	1
Conjunctivitis allergic			
subjects affected / exposed	2 / 22 (9.09%)	1 / 16 (6.25%)	1 / 7 (14.29%)
occurrences (all)	2	1	1
Cataract			
subjects affected / exposed	1 / 22 (4.55%)	1 / 16 (6.25%)	0 / 7 (0.00%)
occurrences (all)	1	1	0
Blepharitis			
subjects affected / exposed	0 / 22 (0.00%)	1 / 16 (6.25%)	0 / 7 (0.00%)
occurrences (all)	0	2	0
Dry eye			
subjects affected / exposed	2 / 22 (9.09%)	0 / 16 (0.00%)	0 / 7 (0.00%)
occurrences (all)	2	0	0
Ocular hyperaemia			

subjects affected / exposed	0 / 22 (0.00%)	0 / 16 (0.00%)	1 / 7 (14.29%)
occurrences (all)	0	0	1
Glaucoma			
subjects affected / exposed	2 / 22 (9.09%)	0 / 16 (0.00%)	0 / 7 (0.00%)
occurrences (all)	2	0	0
Gastrointestinal disorders			
Vomiting			
subjects affected / exposed	3 / 22 (13.64%)	2 / 16 (12.50%)	0 / 7 (0.00%)
occurrences (all)	4	2	0
Lip pain			
subjects affected / exposed	0 / 22 (0.00%)	0 / 16 (0.00%)	1 / 7 (14.29%)
occurrences (all)	0	0	1
Diarrhoea			
subjects affected / exposed	2 / 22 (9.09%)	2 / 16 (12.50%)	1 / 7 (14.29%)
occurrences (all)	2	3	1
Dental caries			
subjects affected / exposed	1 / 22 (4.55%)	1 / 16 (6.25%)	1 / 7 (14.29%)
occurrences (all)	1	1	1
Constipation			
subjects affected / exposed	2 / 22 (9.09%)	1 / 16 (6.25%)	0 / 7 (0.00%)
occurrences (all)	3	1	0
Abdominal pain			
subjects affected / exposed	2 / 22 (9.09%)	1 / 16 (6.25%)	1 / 7 (14.29%)
occurrences (all)	3	1	3
Hepatobiliary disorders			
Jaundice			
subjects affected / exposed	0 / 22 (0.00%)	0 / 16 (0.00%)	1 / 7 (14.29%)
occurrences (all)	0	0	1
Liver disorder			
subjects affected / exposed	0 / 22 (0.00%)	1 / 16 (6.25%)	0 / 7 (0.00%)
occurrences (all)	0	1	0
Skin and subcutaneous tissue disorders			
Alopecia			
subjects affected / exposed	0 / 22 (0.00%)	0 / 16 (0.00%)	1 / 7 (14.29%)
occurrences (all)	0	0	1
Acne			

subjects affected / exposed	2 / 22 (9.09%)	0 / 16 (0.00%)	0 / 7 (0.00%)
occurrences (all)	3	0	0
Dermatitis contact			
subjects affected / exposed	0 / 22 (0.00%)	0 / 16 (0.00%)	1 / 7 (14.29%)
occurrences (all)	0	0	1
Dry skin			
subjects affected / exposed	3 / 22 (13.64%)	0 / 16 (0.00%)	0 / 7 (0.00%)
occurrences (all)	3	0	0
Eczema			
subjects affected / exposed	2 / 22 (9.09%)	2 / 16 (12.50%)	0 / 7 (0.00%)
occurrences (all)	2	2	0
Erythema			
subjects affected / exposed	1 / 22 (4.55%)	2 / 16 (12.50%)	0 / 7 (0.00%)
occurrences (all)	1	2	0
Pruritus			
subjects affected / exposed	1 / 22 (4.55%)	1 / 16 (6.25%)	0 / 7 (0.00%)
occurrences (all)	1	1	0
Hypertrichosis			
subjects affected / exposed	0 / 22 (0.00%)	1 / 16 (6.25%)	1 / 7 (14.29%)
occurrences (all)	0	1	1
Rash			
subjects affected / exposed	1 / 22 (4.55%)	0 / 16 (0.00%)	1 / 7 (14.29%)
occurrences (all)	1	0	1
Skin irritation			
subjects affected / exposed	0 / 22 (0.00%)	0 / 16 (0.00%)	1 / 7 (14.29%)
occurrences (all)	0	0	1
Urticaria			
subjects affected / exposed	0 / 22 (0.00%)	2 / 16 (12.50%)	0 / 7 (0.00%)
occurrences (all)	0	2	0
Rash macular			
subjects affected / exposed	0 / 22 (0.00%)	0 / 16 (0.00%)	1 / 7 (14.29%)
occurrences (all)	0	0	1
Skin haemorrhage			
subjects affected / exposed	0 / 22 (0.00%)	0 / 16 (0.00%)	1 / 7 (14.29%)
occurrences (all)	0	0	1
Endocrine disorders			

Cushing's syndrome			
subjects affected / exposed	0 / 22 (0.00%)	1 / 16 (6.25%)	0 / 7 (0.00%)
occurrences (all)	0	1	0
Musculoskeletal and connective tissue disorders			
Arthralgia			
subjects affected / exposed	3 / 22 (13.64%)	0 / 16 (0.00%)	0 / 7 (0.00%)
occurrences (all)	7	0	0
Osteopenia			
subjects affected / exposed	0 / 22 (0.00%)	1 / 16 (6.25%)	0 / 7 (0.00%)
occurrences (all)	0	1	0
Knee deformity			
subjects affected / exposed	0 / 22 (0.00%)	1 / 16 (6.25%)	0 / 7 (0.00%)
occurrences (all)	0	1	0
Back pain			
subjects affected / exposed	3 / 22 (13.64%)	1 / 16 (6.25%)	0 / 7 (0.00%)
occurrences (all)	3	1	0
Bone pain			
subjects affected / exposed	1 / 22 (4.55%)	1 / 16 (6.25%)	0 / 7 (0.00%)
occurrences (all)	2	1	0
Foot deformity			
subjects affected / exposed	0 / 22 (0.00%)	1 / 16 (6.25%)	0 / 7 (0.00%)
occurrences (all)	0	1	0
Pain in extremity			
subjects affected / exposed	3 / 22 (13.64%)	0 / 16 (0.00%)	0 / 7 (0.00%)
occurrences (all)	3	0	0
Osteoporosis			
subjects affected / exposed	1 / 22 (4.55%)	1 / 16 (6.25%)	0 / 7 (0.00%)
occurrences (all)	1	1	0
Infections and infestations			
Acarodermatitis			
subjects affected / exposed	0 / 22 (0.00%)	1 / 16 (6.25%)	0 / 7 (0.00%)
occurrences (all)	0	1	0
Conjunctivitis			
subjects affected / exposed	1 / 22 (4.55%)	2 / 16 (12.50%)	1 / 7 (14.29%)
occurrences (all)	1	2	1
COVID-19			

subjects affected / exposed	4 / 22 (18.18%)	3 / 16 (18.75%)	2 / 7 (28.57%)
occurrences (all)	4	3	3
Bronchiolitis			
subjects affected / exposed	0 / 22 (0.00%)	1 / 16 (6.25%)	0 / 7 (0.00%)
occurrences (all)	0	1	0
BK virus infection			
subjects affected / exposed	0 / 22 (0.00%)	1 / 16 (6.25%)	0 / 7 (0.00%)
occurrences (all)	0	1	0
Aspergillus infection			
subjects affected / exposed	0 / 22 (0.00%)	0 / 16 (0.00%)	1 / 7 (14.29%)
occurrences (all)	0	0	1
Gastroenteritis viral			
subjects affected / exposed	1 / 22 (4.55%)	0 / 16 (0.00%)	1 / 7 (14.29%)
occurrences (all)	1	0	1
Gastroenteritis			
subjects affected / exposed	0 / 22 (0.00%)	1 / 16 (6.25%)	0 / 7 (0.00%)
occurrences (all)	0	1	0
Fungal skin infection			
subjects affected / exposed	0 / 22 (0.00%)	0 / 16 (0.00%)	1 / 7 (14.29%)
occurrences (all)	0	0	1
Epstein-Barr viraemia			
subjects affected / exposed	0 / 22 (0.00%)	1 / 16 (6.25%)	0 / 7 (0.00%)
occurrences (all)	0	1	0
Cytomegalovirus infection reactivation			
subjects affected / exposed	2 / 22 (9.09%)	2 / 16 (12.50%)	0 / 7 (0.00%)
occurrences (all)	2	2	0
Influenza			
subjects affected / exposed	1 / 22 (4.55%)	2 / 16 (12.50%)	1 / 7 (14.29%)
occurrences (all)	1	2	1
Lower respiratory tract infection			
subjects affected / exposed	0 / 22 (0.00%)	1 / 16 (6.25%)	0 / 7 (0.00%)
occurrences (all)	0	2	0
Parainfluenzae virus infection			
subjects affected / exposed	2 / 22 (9.09%)	0 / 16 (0.00%)	1 / 7 (14.29%)
occurrences (all)	2	0	1

Otitis media			
subjects affected / exposed	0 / 22 (0.00%)	1 / 16 (6.25%)	1 / 7 (14.29%)
occurrences (all)	0	1	1
Oral viral infection			
subjects affected / exposed	0 / 22 (0.00%)	1 / 16 (6.25%)	0 / 7 (0.00%)
occurrences (all)	0	1	0
Onychomycosis			
subjects affected / exposed	0 / 22 (0.00%)	1 / 16 (6.25%)	1 / 7 (14.29%)
occurrences (all)	0	1	1
Nasopharyngitis			
subjects affected / exposed	1 / 22 (4.55%)	3 / 16 (18.75%)	1 / 7 (14.29%)
occurrences (all)	1	3	2
Pneumonia			
subjects affected / exposed	4 / 22 (18.18%)	1 / 16 (6.25%)	0 / 7 (0.00%)
occurrences (all)	4	2	0
Rhinovirus infection			
subjects affected / exposed	0 / 22 (0.00%)	0 / 16 (0.00%)	1 / 7 (14.29%)
occurrences (all)	0	0	2
Skin infection			
subjects affected / exposed	0 / 22 (0.00%)	0 / 16 (0.00%)	1 / 7 (14.29%)
occurrences (all)	0	0	1
Streptococcal infection			
subjects affected / exposed	0 / 22 (0.00%)	0 / 16 (0.00%)	1 / 7 (14.29%)
occurrences (all)	0	0	1
Tonsillitis			
subjects affected / exposed	0 / 22 (0.00%)	0 / 16 (0.00%)	0 / 7 (0.00%)
occurrences (all)	0	0	0
Upper respiratory tract infection			
subjects affected / exposed	1 / 22 (4.55%)	4 / 16 (25.00%)	4 / 7 (57.14%)
occurrences (all)	2	5	11
Urinary tract infection			
subjects affected / exposed	0 / 22 (0.00%)	0 / 16 (0.00%)	1 / 7 (14.29%)
occurrences (all)	0	0	1
Viral infection			
subjects affected / exposed	0 / 22 (0.00%)	0 / 16 (0.00%)	1 / 7 (14.29%)
occurrences (all)	0	0	1

Metabolism and nutrition disorders			
Decreased appetite			
subjects affected / exposed	0 / 22 (0.00%)	1 / 16 (6.25%)	0 / 7 (0.00%)
occurrences (all)	0	1	0
Hypercholesterolaemia			
subjects affected / exposed	2 / 22 (9.09%)	1 / 16 (6.25%)	0 / 7 (0.00%)
occurrences (all)	2	1	0
Hyperkalaemia			
subjects affected / exposed	1 / 22 (4.55%)	2 / 16 (12.50%)	0 / 7 (0.00%)
occurrences (all)	2	2	0
Hyperphosphataemia			
subjects affected / exposed	0 / 22 (0.00%)	1 / 16 (6.25%)	0 / 7 (0.00%)
occurrences (all)	0	1	0
Hypertriglyceridaemia			
subjects affected / exposed	0 / 22 (0.00%)	1 / 16 (6.25%)	0 / 7 (0.00%)
occurrences (all)	0	1	0
Hypoglycaemia			
subjects affected / exposed	2 / 22 (9.09%)	1 / 16 (6.25%)	0 / 7 (0.00%)
occurrences (all)	5	1	0
Hypokalaemia			
subjects affected / exposed	3 / 22 (13.64%)	0 / 16 (0.00%)	0 / 7 (0.00%)
occurrences (all)	4	0	0
Hypomagnesaemia			
subjects affected / exposed	2 / 22 (9.09%)	2 / 16 (12.50%)	0 / 7 (0.00%)
occurrences (all)	3	2	0
Obesity			
subjects affected / exposed	0 / 22 (0.00%)	1 / 16 (6.25%)	0 / 7 (0.00%)
occurrences (all)	0	1	0
Hypozincaemia			
subjects affected / exposed	0 / 22 (0.00%)	1 / 16 (6.25%)	0 / 7 (0.00%)
occurrences (all)	0	1	0
Hypophosphataemia			
subjects affected / exposed	2 / 22 (9.09%)	0 / 16 (0.00%)	0 / 7 (0.00%)
occurrences (all)	2	0	0
Steroid diabetes			

subjects affected / exposed	2 / 22 (9.09%)	0 / 16 (0.00%)	1 / 7 (14.29%)
occurrences (all)	2	0	1
Vitamin D deficiency			
subjects affected / exposed	2 / 22 (9.09%)	1 / 16 (6.25%)	0 / 7 (0.00%)
occurrences (all)	2	2	0

Non-serious adverse events	On-Treatment All subjects	Post-Treatment >=12y - <18y RUX 10mg BID	Post-Treatment >=6y - <12y RUX 5mg BID
Total subjects affected by non-serious adverse events			
subjects affected / exposed	44 / 45 (97.78%)	4 / 22 (18.18%)	2 / 16 (12.50%)
Neoplasms benign, malignant and unspecified (incl cysts and polyps)			
Skin papilloma			
subjects affected / exposed	2 / 45 (4.44%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences (all)	2	0	0
Vascular disorders			
Hypertension			
subjects affected / exposed	7 / 45 (15.56%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences (all)	7	0	0
Hot flush			
subjects affected / exposed	1 / 45 (2.22%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences (all)	1	0	0
General disorders and administration site conditions			
Pyrexia			
subjects affected / exposed	7 / 45 (15.56%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences (all)	10	0	0
Pain			
subjects affected / exposed	3 / 45 (6.67%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences (all)	3	0	0
Oedema peripheral			
subjects affected / exposed	2 / 45 (4.44%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences (all)	3	0	0
Influenza like illness			
subjects affected / exposed	1 / 45 (2.22%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences (all)	1	0	0
Immune system disorders			

Allergy to chemicals			
subjects affected / exposed	1 / 45 (2.22%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences (all)	1	0	0
Reproductive system and breast disorders			
Pruritus genital			
subjects affected / exposed	1 / 45 (2.22%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences (all)	1	0	0
Acquired phimosis			
subjects affected / exposed	1 / 45 (2.22%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences (all)	1	0	0
Respiratory, thoracic and mediastinal disorders			
Acute respiratory failure			
subjects affected / exposed	1 / 45 (2.22%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences (all)	1	0	0
Cough			
subjects affected / exposed	6 / 45 (13.33%)	1 / 22 (4.55%)	0 / 16 (0.00%)
occurrences (all)	10	2	0
Epistaxis			
subjects affected / exposed	1 / 45 (2.22%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences (all)	1	0	0
Pneumothorax			
subjects affected / exposed	1 / 45 (2.22%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences (all)	2	0	0
Oropharyngeal pain			
subjects affected / exposed	2 / 45 (4.44%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences (all)	2	0	0
Productive cough			
subjects affected / exposed	2 / 45 (4.44%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences (all)	2	0	0
Rhinorrhoea			
subjects affected / exposed	1 / 45 (2.22%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences (all)	1	0	0
Psychiatric disorders			
Insomnia			
subjects affected / exposed	3 / 45 (6.67%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences (all)	3	0	0

Investigations			
Alanine aminotransferase increased			
subjects affected / exposed	5 / 45 (11.11%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences (all)	8	0	0
Amylase increased			
subjects affected / exposed	2 / 45 (4.44%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences (all)	2	0	0
Aspartate aminotransferase increased			
subjects affected / exposed	3 / 45 (6.67%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences (all)	5	0	0
Blood creatine phosphokinase increased			
subjects affected / exposed	2 / 45 (4.44%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences (all)	2	0	0
Blood cholesterol increased			
subjects affected / exposed	2 / 45 (4.44%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences (all)	2	0	0
Blood alkaline phosphatase increased			
subjects affected / exposed	2 / 45 (4.44%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences (all)	2	0	0
Blood lactate dehydrogenase increased			
subjects affected / exposed	1 / 45 (2.22%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences (all)	1	0	0
Gamma-glutamyltransferase increased			
subjects affected / exposed	3 / 45 (6.67%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences (all)	4	0	0
Epstein-Barr virus test positive			
subjects affected / exposed	1 / 45 (2.22%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences (all)	1	0	0
C-reactive protein increased			
subjects affected / exposed	2 / 45 (4.44%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences (all)	2	0	0
Hepatic enzyme increased			
subjects affected / exposed	1 / 45 (2.22%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences (all)	1	0	0

Blood magnesium increased			
subjects affected / exposed	1 / 45 (2.22%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences (all)	1	0	0
Neutrophil count decreased			
subjects affected / exposed	9 / 45 (20.00%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences (all)	14	0	0
Lymphocyte count decreased			
subjects affected / exposed	3 / 45 (6.67%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences (all)	5	0	0
Immunoglobulins decreased			
subjects affected / exposed	1 / 45 (2.22%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences (all)	1	0	0
Herpes simplex test positive			
subjects affected / exposed	1 / 45 (2.22%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences (all)	1	0	0
Platelet count decreased			
subjects affected / exposed	7 / 45 (15.56%)	1 / 22 (4.55%)	0 / 16 (0.00%)
occurrences (all)	8	1	0
Serum ferritin increased			
subjects affected / exposed	2 / 45 (4.44%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences (all)	2	0	0
Vascular access device culture positive			
subjects affected / exposed	1 / 45 (2.22%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences (all)	2	0	0
Weight decreased			
subjects affected / exposed	2 / 45 (4.44%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences (all)	2	0	0
Weight increased			
subjects affected / exposed	2 / 45 (4.44%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences (all)	2	0	0
White blood cell count decreased			
subjects affected / exposed	5 / 45 (11.11%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences (all)	7	0	0
Injury, poisoning and procedural complications			

Head injury			
subjects affected / exposed	1 / 45 (2.22%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences (all)	1	0	0
Fall			
subjects affected / exposed	1 / 45 (2.22%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences (all)	1	0	0
Extraskeletal ossification			
subjects affected / exposed	1 / 45 (2.22%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences (all)	1	0	0
Arthropod bite			
subjects affected / exposed	1 / 45 (2.22%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences (all)	1	0	0
Ligament sprain			
subjects affected / exposed	1 / 45 (2.22%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences (all)	1	0	0
Post procedural haemorrhage			
subjects affected / exposed	1 / 45 (2.22%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences (all)	1	0	0
Procedural pain			
subjects affected / exposed	1 / 45 (2.22%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences (all)	1	0	0
Ulna fracture			
subjects affected / exposed	1 / 45 (2.22%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences (all)	1	0	0
Cardiac disorders			
Tachycardia			
subjects affected / exposed	4 / 45 (8.89%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences (all)	5	0	0
Nervous system disorders			
Polyneuropathy			
subjects affected / exposed	1 / 45 (2.22%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences (all)	1	0	0
Headache			
subjects affected / exposed	7 / 45 (15.56%)	1 / 22 (4.55%)	0 / 16 (0.00%)
occurrences (all)	17	1	0
Blood and lymphatic system disorders			

Neutropenia			
subjects affected / exposed	8 / 45 (17.78%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences (all)	10	0	0
Lymphopenia			
subjects affected / exposed	1 / 45 (2.22%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences (all)	2	0	0
Anaemia			
subjects affected / exposed	11 / 45 (24.44%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences (all)	13	0	0
Coagulopathy			
subjects affected / exposed	1 / 45 (2.22%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences (all)	1	0	0
Leukopenia			
subjects affected / exposed	3 / 45 (6.67%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences (all)	4	0	0
Thrombocytopenia			
subjects affected / exposed	5 / 45 (11.11%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences (all)	6	0	0
Ear and labyrinth disorders			
Ear pain			
subjects affected / exposed	1 / 45 (2.22%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences (all)	1	0	0
Ear canal stenosis			
subjects affected / exposed	1 / 45 (2.22%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences (all)	1	0	0
Eye disorders			
Astigmatism			
subjects affected / exposed	1 / 45 (2.22%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences (all)	1	0	0
Conjunctivitis allergic			
subjects affected / exposed	4 / 45 (8.89%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences (all)	4	0	0
Cataract			
subjects affected / exposed	2 / 45 (4.44%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences (all)	2	0	0
Blepharitis			

subjects affected / exposed	1 / 45 (2.22%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences (all)	2	0	0
Dry eye			
subjects affected / exposed	2 / 45 (4.44%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences (all)	2	0	0
Ocular hyperaemia			
subjects affected / exposed	1 / 45 (2.22%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences (all)	1	0	0
Glaucoma			
subjects affected / exposed	2 / 45 (4.44%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences (all)	2	0	0
Gastrointestinal disorders			
Vomiting			
subjects affected / exposed	5 / 45 (11.11%)	1 / 22 (4.55%)	0 / 16 (0.00%)
occurrences (all)	6	1	0
Lip pain			
subjects affected / exposed	1 / 45 (2.22%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences (all)	1	0	0
Diarrhoea			
subjects affected / exposed	5 / 45 (11.11%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences (all)	6	0	0
Dental caries			
subjects affected / exposed	3 / 45 (6.67%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences (all)	3	0	0
Constipation			
subjects affected / exposed	3 / 45 (6.67%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences (all)	4	0	0
Abdominal pain			
subjects affected / exposed	4 / 45 (8.89%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences (all)	7	0	0
Hepatobiliary disorders			
Jaundice			
subjects affected / exposed	1 / 45 (2.22%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences (all)	1	0	0
Liver disorder			

subjects affected / exposed	1 / 45 (2.22%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences (all)	1	0	0
Skin and subcutaneous tissue disorders			
Alopecia			
subjects affected / exposed	1 / 45 (2.22%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences (all)	1	0	0
Acne			
subjects affected / exposed	2 / 45 (4.44%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences (all)	3	0	0
Dermatitis contact			
subjects affected / exposed	1 / 45 (2.22%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences (all)	1	0	0
Dry skin			
subjects affected / exposed	3 / 45 (6.67%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences (all)	3	0	0
Eczema			
subjects affected / exposed	4 / 45 (8.89%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences (all)	4	0	0
Erythema			
subjects affected / exposed	3 / 45 (6.67%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences (all)	3	0	0
Pruritus			
subjects affected / exposed	2 / 45 (4.44%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences (all)	2	0	0
Hypertrichosis			
subjects affected / exposed	2 / 45 (4.44%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences (all)	2	0	0
Rash			
subjects affected / exposed	2 / 45 (4.44%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences (all)	2	0	0
Skin irritation			
subjects affected / exposed	1 / 45 (2.22%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences (all)	1	0	0
Urticaria			
subjects affected / exposed	2 / 45 (4.44%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences (all)	2	0	0

Rash macular			
subjects affected / exposed	1 / 45 (2.22%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences (all)	1	0	0
Skin haemorrhage			
subjects affected / exposed	1 / 45 (2.22%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences (all)	1	0	0
Endocrine disorders			
Cushing's syndrome			
subjects affected / exposed	1 / 45 (2.22%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences (all)	1	0	0
Musculoskeletal and connective tissue disorders			
Arthralgia			
subjects affected / exposed	3 / 45 (6.67%)	1 / 22 (4.55%)	0 / 16 (0.00%)
occurrences (all)	7	1	0
Osteopenia			
subjects affected / exposed	1 / 45 (2.22%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences (all)	1	0	0
Knee deformity			
subjects affected / exposed	1 / 45 (2.22%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences (all)	1	0	0
Back pain			
subjects affected / exposed	4 / 45 (8.89%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences (all)	4	0	0
Bone pain			
subjects affected / exposed	2 / 45 (4.44%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences (all)	3	0	0
Foot deformity			
subjects affected / exposed	1 / 45 (2.22%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences (all)	1	0	0
Pain in extremity			
subjects affected / exposed	3 / 45 (6.67%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences (all)	3	0	0
Osteoporosis			
subjects affected / exposed	2 / 45 (4.44%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences (all)	2	0	0
Infections and infestations			

Acarodermatitis			
subjects affected / exposed	1 / 45 (2.22%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences (all)	1	0	0
Conjunctivitis			
subjects affected / exposed	4 / 45 (8.89%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences (all)	4	0	0
COVID-19			
subjects affected / exposed	9 / 45 (20.00%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences (all)	10	0	0
Bronchiolitis			
subjects affected / exposed	1 / 45 (2.22%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences (all)	1	0	0
BK virus infection			
subjects affected / exposed	1 / 45 (2.22%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences (all)	1	0	0
Aspergillus infection			
subjects affected / exposed	1 / 45 (2.22%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences (all)	1	0	0
Gastroenteritis viral			
subjects affected / exposed	2 / 45 (4.44%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences (all)	2	0	0
Gastroenteritis			
subjects affected / exposed	1 / 45 (2.22%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences (all)	1	0	0
Fungal skin infection			
subjects affected / exposed	1 / 45 (2.22%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences (all)	1	0	0
Epstein-Barr viraemia			
subjects affected / exposed	1 / 45 (2.22%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences (all)	1	0	0
Cytomegalovirus infection reactivation			
subjects affected / exposed	4 / 45 (8.89%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences (all)	4	0	0
Influenza			

subjects affected / exposed	4 / 45 (8.89%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences (all)	4	0	0
Lower respiratory tract infection			
subjects affected / exposed	1 / 45 (2.22%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences (all)	2	0	0
Parainfluenzae virus infection			
subjects affected / exposed	3 / 45 (6.67%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences (all)	3	0	0
Otitis media			
subjects affected / exposed	2 / 45 (4.44%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences (all)	2	0	0
Oral viral infection			
subjects affected / exposed	1 / 45 (2.22%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences (all)	1	0	0
Onychomycosis			
subjects affected / exposed	2 / 45 (4.44%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences (all)	2	0	0
Nasopharyngitis			
subjects affected / exposed	5 / 45 (11.11%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences (all)	6	0	0
Pneumonia			
subjects affected / exposed	5 / 45 (11.11%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences (all)	6	0	0
Rhinovirus infection			
subjects affected / exposed	1 / 45 (2.22%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences (all)	2	0	0
Skin infection			
subjects affected / exposed	1 / 45 (2.22%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences (all)	1	0	0
Streptococcal infection			
subjects affected / exposed	1 / 45 (2.22%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences (all)	1	0	0
Tonsillitis			
subjects affected / exposed	0 / 45 (0.00%)	0 / 22 (0.00%)	1 / 16 (6.25%)
occurrences (all)	0	0	1
Upper respiratory tract infection			

subjects affected / exposed	9 / 45 (20.00%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences (all)	18	0	0
Urinary tract infection			
subjects affected / exposed	1 / 45 (2.22%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences (all)	1	0	0
Viral infection			
subjects affected / exposed	1 / 45 (2.22%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences (all)	1	0	0
Metabolism and nutrition disorders			
Decreased appetite			
subjects affected / exposed	1 / 45 (2.22%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences (all)	1	0	0
Hypercholesterolaemia			
subjects affected / exposed	3 / 45 (6.67%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences (all)	3	0	0
Hyperkalaemia			
subjects affected / exposed	3 / 45 (6.67%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences (all)	4	0	0
Hyperphosphataemia			
subjects affected / exposed	1 / 45 (2.22%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences (all)	1	0	0
Hypertriglyceridaemia			
subjects affected / exposed	1 / 45 (2.22%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences (all)	1	0	0
Hypoglycaemia			
subjects affected / exposed	3 / 45 (6.67%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences (all)	6	0	0
Hypokalaemia			
subjects affected / exposed	3 / 45 (6.67%)	1 / 22 (4.55%)	0 / 16 (0.00%)
occurrences (all)	4	1	0
Hypomagnesaemia			
subjects affected / exposed	4 / 45 (8.89%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences (all)	5	0	0
Obesity			
subjects affected / exposed	1 / 45 (2.22%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences (all)	1	0	0

Hypozincaemia			
subjects affected / exposed	1 / 45 (2.22%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences (all)	1	0	0
Hypophosphataemia			
subjects affected / exposed	2 / 45 (4.44%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences (all)	2	0	0
Steroid diabetes			
subjects affected / exposed	3 / 45 (6.67%)	0 / 22 (0.00%)	0 / 16 (0.00%)
occurrences (all)	3	0	0
Vitamin D deficiency			
subjects affected / exposed	3 / 45 (6.67%)	0 / 22 (0.00%)	1 / 16 (6.25%)
occurrences (all)	4	0	1

Non-serious adverse events	Post-Treatment >=2y - <6y RUX 4mg/m2 BID	Post-Treatment All subjects	
Total subjects affected by non-serious adverse events			
subjects affected / exposed	0 / 7 (0.00%)	6 / 45 (13.33%)	
Neoplasms benign, malignant and unspecified (incl cysts and polyps)			
Skin papilloma			
subjects affected / exposed	0 / 7 (0.00%)	0 / 45 (0.00%)	
occurrences (all)	0	0	
Vascular disorders			
Hypertension			
subjects affected / exposed	0 / 7 (0.00%)	0 / 45 (0.00%)	
occurrences (all)	0	0	
Hot flush			
subjects affected / exposed	0 / 7 (0.00%)	0 / 45 (0.00%)	
occurrences (all)	0	0	
General disorders and administration site conditions			
Pyrexia			
subjects affected / exposed	0 / 7 (0.00%)	0 / 45 (0.00%)	
occurrences (all)	0	0	
Pain			
subjects affected / exposed	0 / 7 (0.00%)	0 / 45 (0.00%)	
occurrences (all)	0	0	
Oedema peripheral			

subjects affected / exposed	0 / 7 (0.00%)	0 / 45 (0.00%)	
occurrences (all)	0	0	
Influenza like illness			
subjects affected / exposed	0 / 7 (0.00%)	0 / 45 (0.00%)	
occurrences (all)	0	0	
Immune system disorders			
Allergy to chemicals			
subjects affected / exposed	0 / 7 (0.00%)	0 / 45 (0.00%)	
occurrences (all)	0	0	
Reproductive system and breast disorders			
Pruritus genital			
subjects affected / exposed	0 / 7 (0.00%)	0 / 45 (0.00%)	
occurrences (all)	0	0	
Acquired phimosis			
subjects affected / exposed	0 / 7 (0.00%)	0 / 45 (0.00%)	
occurrences (all)	0	0	
Respiratory, thoracic and mediastinal disorders			
Acute respiratory failure			
subjects affected / exposed	0 / 7 (0.00%)	0 / 45 (0.00%)	
occurrences (all)	0	0	
Cough			
subjects affected / exposed	0 / 7 (0.00%)	1 / 45 (2.22%)	
occurrences (all)	0	2	
Epistaxis			
subjects affected / exposed	0 / 7 (0.00%)	0 / 45 (0.00%)	
occurrences (all)	0	0	
Pneumothorax			
subjects affected / exposed	0 / 7 (0.00%)	0 / 45 (0.00%)	
occurrences (all)	0	0	
Oropharyngeal pain			
subjects affected / exposed	0 / 7 (0.00%)	0 / 45 (0.00%)	
occurrences (all)	0	0	
Productive cough			
subjects affected / exposed	0 / 7 (0.00%)	0 / 45 (0.00%)	
occurrences (all)	0	0	
Rhinorrhoea			

subjects affected / exposed	0 / 7 (0.00%)	0 / 45 (0.00%)	
occurrences (all)	0	0	
Psychiatric disorders			
Insomnia			
subjects affected / exposed	0 / 7 (0.00%)	0 / 45 (0.00%)	
occurrences (all)	0	0	
Investigations			
Alanine aminotransferase increased			
subjects affected / exposed	0 / 7 (0.00%)	0 / 45 (0.00%)	
occurrences (all)	0	0	
Amylase increased			
subjects affected / exposed	0 / 7 (0.00%)	0 / 45 (0.00%)	
occurrences (all)	0	0	
Aspartate aminotransferase increased			
subjects affected / exposed	0 / 7 (0.00%)	0 / 45 (0.00%)	
occurrences (all)	0	0	
Blood creatine phosphokinase increased			
subjects affected / exposed	0 / 7 (0.00%)	0 / 45 (0.00%)	
occurrences (all)	0	0	
Blood cholesterol increased			
subjects affected / exposed	0 / 7 (0.00%)	0 / 45 (0.00%)	
occurrences (all)	0	0	
Blood alkaline phosphatase increased			
subjects affected / exposed	0 / 7 (0.00%)	0 / 45 (0.00%)	
occurrences (all)	0	0	
Blood lactate dehydrogenase increased			
subjects affected / exposed	0 / 7 (0.00%)	0 / 45 (0.00%)	
occurrences (all)	0	0	
Gamma-glutamyltransferase increased			
subjects affected / exposed	0 / 7 (0.00%)	0 / 45 (0.00%)	
occurrences (all)	0	0	
Epstein-Barr virus test positive			
subjects affected / exposed	0 / 7 (0.00%)	0 / 45 (0.00%)	
occurrences (all)	0	0	

C-reactive protein increased				
subjects affected / exposed	0 / 7 (0.00%)	0 / 45 (0.00%)		
occurrences (all)	0	0		
Hepatic enzyme increased				
subjects affected / exposed	0 / 7 (0.00%)	0 / 45 (0.00%)		
occurrences (all)	0	0		
Blood magnesium increased				
subjects affected / exposed	0 / 7 (0.00%)	0 / 45 (0.00%)		
occurrences (all)	0	0		
Neutrophil count decreased				
subjects affected / exposed	0 / 7 (0.00%)	0 / 45 (0.00%)		
occurrences (all)	0	0		
Lymphocyte count decreased				
subjects affected / exposed	0 / 7 (0.00%)	0 / 45 (0.00%)		
occurrences (all)	0	0		
Immunoglobulins decreased				
subjects affected / exposed	0 / 7 (0.00%)	0 / 45 (0.00%)		
occurrences (all)	0	0		
Herpes simplex test positive				
subjects affected / exposed	0 / 7 (0.00%)	0 / 45 (0.00%)		
occurrences (all)	0	0		
Platelet count decreased				
subjects affected / exposed	0 / 7 (0.00%)	1 / 45 (2.22%)		
occurrences (all)	0	1		
Serum ferritin increased				
subjects affected / exposed	0 / 7 (0.00%)	0 / 45 (0.00%)		
occurrences (all)	0	0		
Vascular access device culture positive				
subjects affected / exposed	0 / 7 (0.00%)	0 / 45 (0.00%)		
occurrences (all)	0	0		
Weight decreased				
subjects affected / exposed	0 / 7 (0.00%)	0 / 45 (0.00%)		
occurrences (all)	0	0		
Weight increased				

subjects affected / exposed	0 / 7 (0.00%)	0 / 45 (0.00%)	
occurrences (all)	0	0	
White blood cell count decreased			
subjects affected / exposed	0 / 7 (0.00%)	0 / 45 (0.00%)	
occurrences (all)	0	0	
Injury, poisoning and procedural complications			
Head injury			
subjects affected / exposed	0 / 7 (0.00%)	0 / 45 (0.00%)	
occurrences (all)	0	0	
Fall			
subjects affected / exposed	0 / 7 (0.00%)	0 / 45 (0.00%)	
occurrences (all)	0	0	
Extraskeletal ossification			
subjects affected / exposed	0 / 7 (0.00%)	0 / 45 (0.00%)	
occurrences (all)	0	0	
Arthropod bite			
subjects affected / exposed	0 / 7 (0.00%)	0 / 45 (0.00%)	
occurrences (all)	0	0	
Ligament sprain			
subjects affected / exposed	0 / 7 (0.00%)	0 / 45 (0.00%)	
occurrences (all)	0	0	
Post procedural haemorrhage			
subjects affected / exposed	0 / 7 (0.00%)	0 / 45 (0.00%)	
occurrences (all)	0	0	
Procedural pain			
subjects affected / exposed	0 / 7 (0.00%)	0 / 45 (0.00%)	
occurrences (all)	0	0	
Ulna fracture			
subjects affected / exposed	0 / 7 (0.00%)	0 / 45 (0.00%)	
occurrences (all)	0	0	
Cardiac disorders			
Tachycardia			
subjects affected / exposed	0 / 7 (0.00%)	0 / 45 (0.00%)	
occurrences (all)	0	0	
Nervous system disorders			

Polyneuropathy			
subjects affected / exposed	0 / 7 (0.00%)	0 / 45 (0.00%)	
occurrences (all)	0	0	
Headache			
subjects affected / exposed	0 / 7 (0.00%)	1 / 45 (2.22%)	
occurrences (all)	0	1	
Blood and lymphatic system disorders			
Neutropenia			
subjects affected / exposed	0 / 7 (0.00%)	0 / 45 (0.00%)	
occurrences (all)	0	0	
Lymphopenia			
subjects affected / exposed	0 / 7 (0.00%)	0 / 45 (0.00%)	
occurrences (all)	0	0	
Anaemia			
subjects affected / exposed	0 / 7 (0.00%)	0 / 45 (0.00%)	
occurrences (all)	0	0	
Coagulopathy			
subjects affected / exposed	0 / 7 (0.00%)	0 / 45 (0.00%)	
occurrences (all)	0	0	
Leukopenia			
subjects affected / exposed	0 / 7 (0.00%)	0 / 45 (0.00%)	
occurrences (all)	0	0	
Thrombocytopenia			
subjects affected / exposed	0 / 7 (0.00%)	0 / 45 (0.00%)	
occurrences (all)	0	0	
Ear and labyrinth disorders			
Ear pain			
subjects affected / exposed	0 / 7 (0.00%)	0 / 45 (0.00%)	
occurrences (all)	0	0	
Ear canal stenosis			
subjects affected / exposed	0 / 7 (0.00%)	0 / 45 (0.00%)	
occurrences (all)	0	0	
Eye disorders			
Astigmatism			
subjects affected / exposed	0 / 7 (0.00%)	0 / 45 (0.00%)	
occurrences (all)	0	0	
Conjunctivitis allergic			

subjects affected / exposed	0 / 7 (0.00%)	0 / 45 (0.00%)	
occurrences (all)	0	0	
Cataract			
subjects affected / exposed	0 / 7 (0.00%)	0 / 45 (0.00%)	
occurrences (all)	0	0	
Blepharitis			
subjects affected / exposed	0 / 7 (0.00%)	0 / 45 (0.00%)	
occurrences (all)	0	0	
Dry eye			
subjects affected / exposed	0 / 7 (0.00%)	0 / 45 (0.00%)	
occurrences (all)	0	0	
Ocular hyperaemia			
subjects affected / exposed	0 / 7 (0.00%)	0 / 45 (0.00%)	
occurrences (all)	0	0	
Glaucoma			
subjects affected / exposed	0 / 7 (0.00%)	0 / 45 (0.00%)	
occurrences (all)	0	0	
Gastrointestinal disorders			
Vomiting			
subjects affected / exposed	0 / 7 (0.00%)	1 / 45 (2.22%)	
occurrences (all)	0	1	
Lip pain			
subjects affected / exposed	0 / 7 (0.00%)	0 / 45 (0.00%)	
occurrences (all)	0	0	
Diarrhoea			
subjects affected / exposed	0 / 7 (0.00%)	0 / 45 (0.00%)	
occurrences (all)	0	0	
Dental caries			
subjects affected / exposed	0 / 7 (0.00%)	0 / 45 (0.00%)	
occurrences (all)	0	0	
Constipation			
subjects affected / exposed	0 / 7 (0.00%)	0 / 45 (0.00%)	
occurrences (all)	0	0	
Abdominal pain			
subjects affected / exposed	0 / 7 (0.00%)	0 / 45 (0.00%)	
occurrences (all)	0	0	

Hepatobiliary disorders			
Jaundice			
subjects affected / exposed	0 / 7 (0.00%)	0 / 45 (0.00%)	
occurrences (all)	0	0	
Liver disorder			
subjects affected / exposed	0 / 7 (0.00%)	0 / 45 (0.00%)	
occurrences (all)	0	0	
Skin and subcutaneous tissue disorders			
Alopecia			
subjects affected / exposed	0 / 7 (0.00%)	0 / 45 (0.00%)	
occurrences (all)	0	0	
Acne			
subjects affected / exposed	0 / 7 (0.00%)	0 / 45 (0.00%)	
occurrences (all)	0	0	
Dermatitis contact			
subjects affected / exposed	0 / 7 (0.00%)	0 / 45 (0.00%)	
occurrences (all)	0	0	
Dry skin			
subjects affected / exposed	0 / 7 (0.00%)	0 / 45 (0.00%)	
occurrences (all)	0	0	
Eczema			
subjects affected / exposed	0 / 7 (0.00%)	0 / 45 (0.00%)	
occurrences (all)	0	0	
Erythema			
subjects affected / exposed	0 / 7 (0.00%)	0 / 45 (0.00%)	
occurrences (all)	0	0	
Pruritus			
subjects affected / exposed	0 / 7 (0.00%)	0 / 45 (0.00%)	
occurrences (all)	0	0	
Hypertrichosis			
subjects affected / exposed	0 / 7 (0.00%)	0 / 45 (0.00%)	
occurrences (all)	0	0	
Rash			
subjects affected / exposed	0 / 7 (0.00%)	0 / 45 (0.00%)	
occurrences (all)	0	0	
Skin irritation			

subjects affected / exposed	0 / 7 (0.00%)	0 / 45 (0.00%)	
occurrences (all)	0	0	
Urticaria			
subjects affected / exposed	0 / 7 (0.00%)	0 / 45 (0.00%)	
occurrences (all)	0	0	
Rash macular			
subjects affected / exposed	0 / 7 (0.00%)	0 / 45 (0.00%)	
occurrences (all)	0	0	
Skin haemorrhage			
subjects affected / exposed	0 / 7 (0.00%)	0 / 45 (0.00%)	
occurrences (all)	0	0	
Endocrine disorders			
Cushing's syndrome			
subjects affected / exposed	0 / 7 (0.00%)	0 / 45 (0.00%)	
occurrences (all)	0	0	
Musculoskeletal and connective tissue disorders			
Arthralgia			
subjects affected / exposed	0 / 7 (0.00%)	1 / 45 (2.22%)	
occurrences (all)	0	1	
Osteopenia			
subjects affected / exposed	0 / 7 (0.00%)	0 / 45 (0.00%)	
occurrences (all)	0	0	
Knee deformity			
subjects affected / exposed	0 / 7 (0.00%)	0 / 45 (0.00%)	
occurrences (all)	0	0	
Back pain			
subjects affected / exposed	0 / 7 (0.00%)	0 / 45 (0.00%)	
occurrences (all)	0	0	
Bone pain			
subjects affected / exposed	0 / 7 (0.00%)	0 / 45 (0.00%)	
occurrences (all)	0	0	
Foot deformity			
subjects affected / exposed	0 / 7 (0.00%)	0 / 45 (0.00%)	
occurrences (all)	0	0	
Pain in extremity			

subjects affected / exposed	0 / 7 (0.00%)	0 / 45 (0.00%)	
occurrences (all)	0	0	
Osteoporosis			
subjects affected / exposed	0 / 7 (0.00%)	0 / 45 (0.00%)	
occurrences (all)	0	0	
Infections and infestations			
Acarodermatitis			
subjects affected / exposed	0 / 7 (0.00%)	0 / 45 (0.00%)	
occurrences (all)	0	0	
Conjunctivitis			
subjects affected / exposed	0 / 7 (0.00%)	0 / 45 (0.00%)	
occurrences (all)	0	0	
COVID-19			
subjects affected / exposed	0 / 7 (0.00%)	0 / 45 (0.00%)	
occurrences (all)	0	0	
Bronchiolitis			
subjects affected / exposed	0 / 7 (0.00%)	0 / 45 (0.00%)	
occurrences (all)	0	0	
BK virus infection			
subjects affected / exposed	0 / 7 (0.00%)	0 / 45 (0.00%)	
occurrences (all)	0	0	
Aspergillus infection			
subjects affected / exposed	0 / 7 (0.00%)	0 / 45 (0.00%)	
occurrences (all)	0	0	
Gastroenteritis viral			
subjects affected / exposed	0 / 7 (0.00%)	0 / 45 (0.00%)	
occurrences (all)	0	0	
Gastroenteritis			
subjects affected / exposed	0 / 7 (0.00%)	0 / 45 (0.00%)	
occurrences (all)	0	0	
Fungal skin infection			
subjects affected / exposed	0 / 7 (0.00%)	0 / 45 (0.00%)	
occurrences (all)	0	0	
Epstein-Barr viraemia			
subjects affected / exposed	0 / 7 (0.00%)	0 / 45 (0.00%)	
occurrences (all)	0	0	

Cytomegalovirus infection reactivation				
subjects affected / exposed	0 / 7 (0.00%)	0 / 45 (0.00%)		
occurrences (all)	0	0		
Influenza				
subjects affected / exposed	0 / 7 (0.00%)	0 / 45 (0.00%)		
occurrences (all)	0	0		
Lower respiratory tract infection				
subjects affected / exposed	0 / 7 (0.00%)	0 / 45 (0.00%)		
occurrences (all)	0	0		
Parainfluenzae virus infection				
subjects affected / exposed	0 / 7 (0.00%)	0 / 45 (0.00%)		
occurrences (all)	0	0		
Otitis media				
subjects affected / exposed	0 / 7 (0.00%)	0 / 45 (0.00%)		
occurrences (all)	0	0		
Oral viral infection				
subjects affected / exposed	0 / 7 (0.00%)	0 / 45 (0.00%)		
occurrences (all)	0	0		
Onychomycosis				
subjects affected / exposed	0 / 7 (0.00%)	0 / 45 (0.00%)		
occurrences (all)	0	0		
Nasopharyngitis				
subjects affected / exposed	0 / 7 (0.00%)	0 / 45 (0.00%)		
occurrences (all)	0	0		
Pneumonia				
subjects affected / exposed	0 / 7 (0.00%)	0 / 45 (0.00%)		
occurrences (all)	0	0		
Rhinovirus infection				
subjects affected / exposed	0 / 7 (0.00%)	0 / 45 (0.00%)		
occurrences (all)	0	0		
Skin infection				
subjects affected / exposed	0 / 7 (0.00%)	0 / 45 (0.00%)		
occurrences (all)	0	0		
Streptococcal infection				

subjects affected / exposed	0 / 7 (0.00%)	0 / 45 (0.00%)	
occurrences (all)	0	0	
Tonsillitis			
subjects affected / exposed	0 / 7 (0.00%)	1 / 45 (2.22%)	
occurrences (all)	0	1	
Upper respiratory tract infection			
subjects affected / exposed	0 / 7 (0.00%)	0 / 45 (0.00%)	
occurrences (all)	0	0	
Urinary tract infection			
subjects affected / exposed	0 / 7 (0.00%)	0 / 45 (0.00%)	
occurrences (all)	0	0	
Viral infection			
subjects affected / exposed	0 / 7 (0.00%)	0 / 45 (0.00%)	
occurrences (all)	0	0	
Metabolism and nutrition disorders			
Decreased appetite			
subjects affected / exposed	0 / 7 (0.00%)	0 / 45 (0.00%)	
occurrences (all)	0	0	
Hypercholesterolaemia			
subjects affected / exposed	0 / 7 (0.00%)	0 / 45 (0.00%)	
occurrences (all)	0	0	
Hyperkalaemia			
subjects affected / exposed	0 / 7 (0.00%)	0 / 45 (0.00%)	
occurrences (all)	0	0	
Hyperphosphataemia			
subjects affected / exposed	0 / 7 (0.00%)	0 / 45 (0.00%)	
occurrences (all)	0	0	
Hypertriglyceridaemia			
subjects affected / exposed	0 / 7 (0.00%)	0 / 45 (0.00%)	
occurrences (all)	0	0	
Hypoglycaemia			
subjects affected / exposed	0 / 7 (0.00%)	0 / 45 (0.00%)	
occurrences (all)	0	0	
Hypokalaemia			
subjects affected / exposed	0 / 7 (0.00%)	1 / 45 (2.22%)	
occurrences (all)	0	1	

Hypomagnesaemia			
subjects affected / exposed	0 / 7 (0.00%)	0 / 45 (0.00%)	
occurrences (all)	0	0	
Obesity			
subjects affected / exposed	0 / 7 (0.00%)	0 / 45 (0.00%)	
occurrences (all)	0	0	
Hypozincaemia			
subjects affected / exposed	0 / 7 (0.00%)	0 / 45 (0.00%)	
occurrences (all)	0	0	
Hypophosphataemia			
subjects affected / exposed	0 / 7 (0.00%)	0 / 45 (0.00%)	
occurrences (all)	0	0	
Steroid diabetes			
subjects affected / exposed	0 / 7 (0.00%)	0 / 45 (0.00%)	
occurrences (all)	0	0	
Vitamin D deficiency			
subjects affected / exposed	0 / 7 (0.00%)	1 / 45 (2.22%)	
occurrences (all)	0	1	

More information

Substantial protocol amendments (globally)

Were there any global substantial amendments to the protocol? Yes

Date	Amendment
19 November 2020	Update of the guidance regarding the management of ruxolitinib. Update the inclusion criteria to allow for nasogastric tube administration of the oral. pediatric formulation Ruxolitinib tapering management clarification Guidance inclusion for the assessment of organ involvement and response. Ruxolitinib post-trial access requirements clarification. Contraception guidelines and pregnancy reporting requirements update. An assessment of benefit, risk and trial integrity related to SARS-CoV-2 virus and the COVID-19 pandemic was conducted and determined no substantial risk for subject safety or additional measures regarding study design or conduct was warranted.
09 September 2022	Public health emergency disruption proofing language inclusion. Clarification on ruxolitinib treatment management and to subject withdrawal of consent.

Notes:

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.

Due to EudraCT system limitations, which EMA is aware of, data using 999 as data points in this record are not an accurate representation of the clinical trial results.
Please use https://www.novctrd.com for complete trial results.

Notes: