Clinical trial results:
A Multicenter, Open-Label Extension Study of WVE-210201 in Patients Previously Enrolled in WVE-DMDX51-001

Summary

EudraCT number	2018-000975-34
Trial protocol	GB FR NL BE IT
Global end of trial date	20 January 2020

Results information

Result version number	v1 (current)
This version publication date	13 September 2020
First version publication date	13 September 2020

Trial information

Trial identification

Sponsor protocol code	WVE-DMDX51-002

Additional study identifiers

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

Notes:

Sponsors

Sponsor organisation name	Wave Life Sciences UK Limited
Sponsor organisation address	1 Chamberlain Square CS, Birmingham, United Kingdom, B3 3AX
Public contact	Chief Medical Officer, Wave Life Sciences, +617 949-2900, info@wavelifesci.com
Scientific contact	Chief Medical Officer, Wave Life Sciences, +617 949-2900, info@wavelifesci.com

Notes:

Paediatric regulatory details

Is trial part of an agreed paediatric investigation plan (PIP)	No
Does article 45 of REGULATION (EC) No 1901/2006 apply to this trial?	No
Does article 46 of REGULATION (EC) No 1901/2006 apply to this trial?	No

Notes:

Results analysis stage

Analysis stage	Final
Date of interim/final analysis	18 February 2020
Is this the analysis of the primary completion data?	No

Global end of trial reached?	Yes
Global end of trial date	20 January 2020
Was the trial ended prematurely?	No

Notes:

General information about the trial

Main objective of the trial:

To evaluate the safety and tolerability of WVE-210201

Protection of trial subjects:

Written informed consent from each patient or patient's parent(s) or legal guardian(s), if applicable, and written assent from each patient, if applicable, were obtained before any study-specific screening or baseline period evaluations were performed. The anonymity of participating patients was maintained to the extent required by applicable laws and in accordance with current HIPAA standards. This study was designed and monitored in accordance with Sponsor procedures, which complied with the ethical principles of Good Clinical Practice (GCP) as required by the major regulatory authorities, and in accordance with the Declaration of Helsinki.

The unblinded, independent Safety Monitoring Committee (SMC) reviewed aggregate safety data periodically and unblinded, aggregate safety data periodically and on an ad hoc basis should any emergent safety concerns have arisen during the course of the study. Recommendations based on these reviews were to be provided to the Sponsor

Background therapy: -
Evidence for comparator: -

Actual start date of recruitment	14 August 2018
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	Canada: 1
Country: Number of subjects enrolled	Italy: 5
Country: Number of subjects enrolled	United States: 10
Country: Number of subjects enrolled	United Kingdom: 8
Country: Number of subjects enrolled	Belgium: 8
Country: Number of subjects enrolled	France: 5
Worldwide total number of subjects	37
EEA total number of subjects	26

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)	33
Adolescents (12-17 years)	4
Adults (18-64 years)	0
From 65 to 84 years	0
85 years and over	0

Subject disposition

Recruitment

Recruitment details:

This study was conducted in 6 countries (Belgium, France, Italy, United Kingdom, Canada and United States from 14 August 2018 to 20 January 2020).

Pre-assignment

Screening details:

The patients successfully completed the Phase I study (WVE-DMDX51-001) were eligible to participate in this open-label extension study (OLE study). They were re-evaluated for certain eligibility criteria. Patients started screening for this OLE study after a minimum of 2 weeks from the last follow-up visit in the Phase I study.

Period 1

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

Blinding implementation details:

This is an open-label study.

Arms

Are arms mutually exclusive?	Yes
Arm title	1 mg/kg WVE-210201

Arm description:

Dose at enrollment, 1 mg/kg WVE-210201 administered via IV infusion

Arm type	Experimental
Investigational medicinal product name	suvodirsen
Investigational medicinal product code	WVE-210201
Other name	
Pharmaceutical forms	Powder for infusion, Solution for infusion
Routes of administration	Intravenous use

Dosage and administration details:

Patients received weekly IV infusions of suvodirsen. WVE-210201 was provided either as a lyophilized powder or as an isotonic solution for dilution for infusion.

Arm title	2 mg/kg WVE-210201

Arm description:

Dose at enrollment, 2 mg/kg WVE-210201 administered via IV infusion

Arm type	Experimental
Investigational medicinal product name	suvodirsen
Investigational medicinal product code	WVE-210201
Other name	
Pharmaceutical forms	Solution for infusion, Powder for infusion
Routes of administration	Intravenous use

Dosage and administration details:

Patients received weekly IV infusions of suvodirsen. WVE-210201 was provided either as a lyophilized powder or as an isotonic solution for dilution for infusion.

Arm title	3.5 mg/kg WVE-210201

Arm description:

Dose at enrollment, 3.5 mg/kg WVE-210201 administered via IV infusion

Arm type	Experimental

Investigational medicinal product name	suvodirsen
Investigational medicinal product code	WVE-210201
Other name	
Pharmaceutical forms	Solution for infusion, Powder for infusion
Routes of administration	Intravenous use

Dosage and administration details:

Patients received weekly IV infusions of suvodirsen. WVE-210201 was provided either as a lyophilized powder or as an isotonic solution for dilution for infusion.

Arm title	5 mg/kg WVE-210201

Arm description:

Dose at enrollment, 5 mg/kg WVE-210201 administered via IV infusion

Arm type	Experimental
Investigational medicinal product name	suvodirsen
Investigational medicinal product code	WVE-210201
Other name	
Pharmaceutical forms	Powder for infusion, Solution for infusion
Routes of administration	Intravenous use

Dosage and administration details:

Patients received weekly IV infusions of suvodirsen. WVE-210201 was provided either as a lyophilized powder or as an isotonic solution for dilution for infusion.

Number of subjects in period 1	1 mg/kg WVE-210201	2 mg/kg WVE-210201	3.5 mg/kg WVE-210201
Started	14	5	5
Completed Dose Modification Visit	13	5	0
Target Dose 3.5 mg/kg	1	4	0
Target Dose 5 mg/kg	12	1	0
Completed	0	0	0
Not completed	14	5	5
Physician decision	1	-	-
Study Terminated by Sponsor	13	5	5

Number of subjects in period 1	5 mg/kg WVE-210201
Started	13
Completed Dose Modification Visit	0
Target Dose 3.5 mg/kg	0
Target Dose 5 mg/kg	0
Completed	0
Not completed	13
Physician decision	-
Study Terminated by Sponsor	13

Baseline characteristics

Reporting groups	
Reporting group title	1 mg/kg WVE-210201
Reporting group description:	
Dose at enrollment, 1 mg/kg WVE-210201 administered via IV infusion	
Reporting group title	2 mg/kg WVE-210201
Reporting group description:	
Dose at enrollment, 2 mg/kg WVE-210201 administered via IV infusion	
Reporting group title	3.5 mg/kg WVE-210201
Reporting group description:	
Dose at enrollment, 3.5 mg/kg WVE-210201 administered via IV infusion	
Reporting group title	5 mg/kg WVE-210201
Reporting group description:	
Dose at enrollment, 5 mg/kg WVE-210201 administered via IV infusion	

Reporting group values	1 mg/kg WVE-210201	2 mg/kg WVE-210201	3.5 mg/kg WVE-210201
Number of subjects	14	5	5
Age categorical Units: Subjects			
In utero	0	0	0
Preterm newborn infants (gestational age < 37 wks)	0	0	0
Newborns (0-27 days)	0	0	0
Infants and toddlers (28 days-23 months)	0	0	0
Children (2-11 years)	12	4	5
Adolescents (12-17 years)	2	1	0
Adults (18-64 years)	0	0	0
From 65-84 years	0	0	0
85 years and over	0	0	0
Gender categorical Units: Subjects			
Female	0	0	0
Male	14	5	5

Reporting group values	5 mg/kg WVE-210201	Total	
Number of subjects	13	37	
Age categorical Units: Subjects			
In utero	0	0	
Preterm newborn infants (gestational age < 37 wks)	0	0	
Newborns (0-27 days)	0	0	
Infants and toddlers (28 days-23 months)	0	0	
Children (2-11 years)	12	33	
Adolescents (12-17 years)	1	4	
Adults (18-64 years)	0	0	

From 65-84 years	0	0	
85 years and over	0	0	

Gender categorical Units: Subjects			
Female	0	0	
Male	13	37	

Subject analysis sets

Subject analysis set title	Safety population
Subject analysis set type	Safety analysis

Subject analysis set description:

All patients who received at least 1 dose of WVE-210201

Reporting group values	Safety population		
Number of subjects	37		
Age categorical Units: Subjects			
In utero	0		
Preterm newborn infants (gestational age < 37 wks)	0		
Newborns (0-27 days)	0		
Infants and toddlers (28 days-23 months)	0		
Children (2-11 years)	33		
Adolescents (12-17 years)	4		
Adults (18-64 years)	0		
From 65-84 years	0		
85 years and over	0		
Gender categorical Units: Subjects			
Female	0		
Male	37		

End points

End points reporting groups

Reporting group title	1 mg/kg WVE-210201
Reporting group description:	
Dose at enrollment, 1 mg/kg WVE-210201 administered via IV infusion	
Reporting group title	2 mg/kg WVE-210201
Reporting group description:	
Dose at enrollment, 2 mg/kg WVE-210201 administered via IV infusion	
Reporting group title	3.5 mg/kg WVE-210201
Reporting group description:	
Dose at enrollment, 3.5 mg/kg WVE-210201 administered via IV infusion	
Reporting group title	5 mg/kg WVE-210201
Reporting group description:	
Dose at enrollment, 5 mg/kg WVE-210201 administered via IV infusion	
Subject analysis set title	Safety population
Subject analysis set type	Safety analysis
Subject analysis set description:	
All patients who received at least 1 dose of WVE-210201	

Primary: Number of Patients who Experienced a Serious TEAE

End point title	Number of Patients who Experienced a Serious TEAE[1]

End point description:

An SAE was defined as any event that resulted in death, was immediately life threatening, required inpatient hospitalization or prolongation of existing hospitalization, resulted in persistent or significant disability/incapacity, or was a congenital anomaly/birth defect not present at Screening. Important medical events that did not result in death, were life threatening, or required hospitalization were considered SAEs when, based upon appropriate medical judgment, they jeopardized the patient or required medical or surgical intervention to prevent one of the outcomes listed in this definition. No patients were withdrawn due to a serious or intolerable AE that in the Investigator's opinion required discontinuation of study drug. 3 patients experienced severe adverse events (2 in 3.5 mg/kg WVE-210201 arm and 1 in 5 mg/kg WVE-210201 arm). This may include patients whose doses were modified.

End point type	Primary

End point timeframe:

Day 1 to Early Termination

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 has been used.

End point values	1 mg/kg WVE-210201	2 mg/kg WVE-210201	3.5 mg/kg WVE-210201	5 mg/kg WVE-210201
Subject group type	Reporting group	Reporting group	Reporting group	Reporting group
Number of subjects analysed	14	5	5	13
Units: Subjects				
number (not applicable)				
Number of Patients who Experienced an SAE	0	0	1	5

Statistical analyses

No statistical analyses for this end point

Adverse events

Adverse events information

Timeframe for reporting adverse events:

Day 1 to Early Termination

Adverse event reporting additional description:

For the patients who had a dose modification during the course of the study, events that occurred prior to dose modification are counted in the dose arm that the patient received at the time of enrollment, and events that occurred after dose modification are counted in the dose arm that the patient received after dose modification.

Assessment type	Systematic

Dictionary used

Dictionary name	MedDRA
Dictionary version	21.0

Reporting groups

Reporting group title	1 mg/kg WVE-210201
Reporting group description:	
1 mg/kg WVE-210201 administered via IV infusion	
Reporting group title	2 mg/kg WVE-210201
Reporting group description:	
2 mg/kg WVE-210201 administered via IV infusion	
Reporting group title	3.5 mg/kg WVE-210201
Reporting group description:	
3.5 mg/kg WVE-210201 administered via IV infusion.	
Reporting group title	5 mg/kg WVE-210201
Reporting group description:	
5 mg/kg WVE-210201 administered via IV infusion	

Serious adverse events	1 mg/kg WVE-210201	2 mg/kg WVE-210201	3.5 mg/kg WVE-210201
Total subjects affected by serious adverse events			
subjects affected / exposed	0 / 14 (0.00%)	0 / 5 (0.00%)	1 / 10 (10.00%)
number of deaths (all causes)	0	0	0
number of deaths resulting from adverse events	0	0	0
Investigations			
Blood bilirubin increased			
subjects affected / exposed	0 / 14 (0.00%)	0 / 5 (0.00%)	1 / 10 (10.00%)
occurrences causally related to treatment / all	0 / 0	0 / 0	1 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Gamma-glutamyltransferase increased			

subjects affected / exposed	0 / 14 (0.00%)	0 / 5 (0.00%)	1 / 10 (10.00%)
occurrences causally related to treatment / all	0 / 0	0 / 0	1 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Glutamate dehydrogenase increased			
subjects affected / exposed	0 / 14 (0.00%)	0 / 5 (0.00%)	1 / 10 (10.00%)
occurrences causally related to treatment / all	0 / 0	0 / 0	1 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Injury, poisoning and procedural complications			
Lumbar vertebral fracture			
subjects affected / exposed	0 / 14 (0.00%)	0 / 5 (0.00%)	0 / 10 (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
Cardiac disorders			
Tachycardia			
subjects affected / exposed	0 / 14 (0.00%)	0 / 5 (0.00%)	0 / 10 (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
Sinus tachycardia			
subjects affected / exposed	0 / 14 (0.00%)	0 / 5 (0.00%)	0 / 10 (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
General disorders and administration site conditions			
Pyrexia			
subjects affected / exposed	0 / 14 (0.00%)	0 / 5 (0.00%)	0 / 10 (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
Gastrointestinal disorders			
Nausea			
subjects affected / exposed	0 / 14 (0.00%)	0 / 5 (0.00%)	0 / 10 (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
Abdominal pain			

subjects affected / exposed	0 / 14 (0.00%)	0 / 5 (0.00%)	0 / 10 (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
Vomiting			
subjects affected / exposed	0 / 14 (0.00%)	0 / 5 (0.00%)	0 / 10 (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

Serious adverse events	5 mg/kg WVE-210201		
Total subjects affected by serious adverse events			
subjects affected / exposed	5 / 26 (19.23%)		
number of deaths (all causes)	0		
number of deaths resulting from adverse events	0		
Investigations			
Blood bilirubin increased			
subjects affected / exposed	0 / 26 (0.00%)		
occurrences causally related to treatment / all	0 / 0		
deaths causally related to treatment / all	0 / 0		
Gamma-glutamyltransferase increased			
subjects affected / exposed	0 / 26 (0.00%)		
occurrences causally related to treatment / all	0 / 0		
deaths causally related to treatment / all	0 / 0		
Glutamate dehydrogenase increased			
subjects affected / exposed	0 / 26 (0.00%)		
occurrences causally related to treatment / all	0 / 0		
deaths causally related to treatment / all	0 / 0		
Injury, poisoning and procedural complications			
Lumbar vertebral fracture			
subjects affected / exposed	1 / 26 (3.85%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Cardiac disorders			
Tachycardia			

subjects affected / exposed	1 / 26 (3.85%)		
occurrences causally related to treatment / all	1 / 1		
deaths causally related to treatment / all	0 / 0		
Sinus tachycardia			
subjects affected / exposed	1 / 26 (3.85%)		
occurrences causally related to treatment / all	1 / 1		
deaths causally related to treatment / all	0 / 0		
General disorders and administration site conditions			
Pyrexia			
subjects affected / exposed	4 / 26 (15.38%)		
occurrences causally related to treatment / all	4 / 4		
deaths causally related to treatment / all	0 / 0		
Gastrointestinal disorders			
Nausea			
subjects affected / exposed	2 / 26 (7.69%)		
occurrences causally related to treatment / all	2 / 2		
deaths causally related to treatment / all	0 / 0		
Abdominal pain			
subjects affected / exposed	1 / 26 (3.85%)		
occurrences causally related to treatment / all	1 / 1		
deaths causally related to treatment / all	0 / 0		
Vomiting			
subjects affected / exposed	1 / 26 (3.85%)		
occurrences causally related to treatment / all	1 / 1		
deaths causally related to treatment / all	0 / 0		

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

Non-serious adverse events	1 mg/kg WVE-210201	2 mg/kg WVE-210201	3.5 mg/kg WVE-210201
Total subjects affected by non-serious adverse events			
subjects affected / exposed	14 / 14 (100.00%)	5 / 5 (100.00%)	10 / 10 (100.00%)
Vascular disorders			
Flushing			

subjects affected / exposed	1 / 14 (7.14%)	0 / 5 (0.00%)	1 / 10 (10.00%)
occurrences (all)	1	0	5
Haematoma			
subjects affected / exposed	0 / 14 (0.00%)	0 / 5 (0.00%)	1 / 10 (10.00%)
occurrences (all)	0	0	1
Hypertension			
subjects affected / exposed	0 / 14 (0.00%)	0 / 5 (0.00%)	0 / 10 (0.00%)
occurrences (all)	0	0	0
Pallor			
subjects affected / exposed	0 / 14 (0.00%)	0 / 5 (0.00%)	0 / 10 (0.00%)
occurrences (all)	0	0	0
General disorders and administration site conditions			
Catheter site erythema			
subjects affected / exposed	0 / 14 (0.00%)	0 / 5 (0.00%)	1 / 10 (10.00%)
occurrences (all)	0	0	1
Chest discomfort			
subjects affected / exposed	0 / 14 (0.00%)	0 / 5 (0.00%)	0 / 10 (0.00%)
occurrences (all)	0	0	0
Chest pain			
subjects affected / exposed	1 / 14 (7.14%)	0 / 5 (0.00%)	0 / 10 (0.00%)
occurrences (all)	1	0	0
Chills			
subjects affected / exposed	0 / 14 (0.00%)	0 / 5 (0.00%)	2 / 10 (20.00%)
occurrences (all)	0	0	4
Fatigue			
subjects affected / exposed	0 / 14 (0.00%)	0 / 5 (0.00%)	2 / 10 (20.00%)
occurrences (all)	0	0	3
Feeling abnormal			
subjects affected / exposed	0 / 14 (0.00%)	0 / 5 (0.00%)	0 / 10 (0.00%)
occurrences (all)	0	0	0
Feeling cold			
subjects affected / exposed	0 / 14 (0.00%)	0 / 5 (0.00%)	0 / 10 (0.00%)
occurrences (all)	0	0	0
Hyperthermia			

subjects affected / exposed	1 / 14 (7.14%)	0 / 5 (0.00%)	0 / 10 (0.00%)
occurrences (all)	1	0	0
Infusion site erythema			
subjects affected / exposed	0 / 14 (0.00%)	0 / 5 (0.00%)	0 / 10 (0.00%)
occurrences (all)	0	0	0
Infusion site papule			
subjects affected / exposed	0 / 14 (0.00%)	0 / 5 (0.00%)	0 / 10 (0.00%)
occurrences (all)	0	0	0
Malaise			
subjects affected / exposed	0 / 14 (0.00%)	0 / 5 (0.00%)	0 / 10 (0.00%)
occurrences (all)	0	0	0
Non-cardiac chest pain			
subjects affected / exposed	0 / 14 (0.00%)	0 / 5 (0.00%)	1 / 10 (10.00%)
occurrences (all)	0	0	1
Pain			
subjects affected / exposed	1 / 14 (7.14%)	0 / 5 (0.00%)	0 / 10 (0.00%)
occurrences (all)	1	0	0
Peripheral swelling			
subjects affected / exposed	1 / 14 (7.14%)	0 / 5 (0.00%)	0 / 10 (0.00%)
occurrences (all)	2	0	0
Pyrexia			
subjects affected / exposed	1 / 14 (7.14%)	0 / 5 (0.00%)	6 / 10 (60.00%)
occurrences (all)	1	0	29
Immune system disorders			
Allergy to arthropod bite			
subjects affected / exposed	0 / 14 (0.00%)	0 / 5 (0.00%)	1 / 10 (10.00%)
occurrences (all)	0	0	1
Seasonal allergy			
subjects affected / exposed	1 / 14 (7.14%)	0 / 5 (0.00%)	1 / 10 (10.00%)
occurrences (all)	1	0	1
Reproductive system and breast disorders			
Pelvic pain			
subjects affected / exposed	0 / 14 (0.00%)	0 / 5 (0.00%)	0 / 10 (0.00%)
occurrences (all)	0	0	0
Respiratory, thoracic and mediastinal disorders			

Cough			
subjects affected / exposed	0 / 14 (0.00%)	0 / 5 (0.00%)	3 / 10 (30.00%)
occurrences (all)	0	0	3
Dyspnoea			
subjects affected / exposed	1 / 14 (7.14%)	0 / 5 (0.00%)	1 / 10 (10.00%)
occurrences (all)	1	0	1
Epistaxis			
subjects affected / exposed	0 / 14 (0.00%)	0 / 5 (0.00%)	1 / 10 (10.00%)
occurrences (all)	0	0	1
Hypoxia			
subjects affected / exposed	0 / 14 (0.00%)	0 / 5 (0.00%)	0 / 10 (0.00%)
occurrences (all)	0	0	0
Nasal congestion			
subjects affected / exposed	2 / 14 (14.29%)	0 / 5 (0.00%)	0 / 10 (0.00%)
occurrences (all)	2	0	0
Oropharyngeal pain			
subjects affected / exposed	0 / 14 (0.00%)	0 / 5 (0.00%)	0 / 10 (0.00%)
occurrences (all)	0	0	0
Rhinorrhoea			
subjects affected / exposed	0 / 14 (0.00%)	1 / 5 (20.00%)	2 / 10 (20.00%)
occurrences (all)	0	1	2
Sneezing			
subjects affected / exposed	0 / 14 (0.00%)	0 / 5 (0.00%)	1 / 10 (10.00%)
occurrences (all)	0	0	1
Sputum discoloured			
subjects affected / exposed	0 / 14 (0.00%)	0 / 5 (0.00%)	1 / 10 (10.00%)
occurrences (all)	0	0	1
Throat irritation			
subjects affected / exposed	0 / 14 (0.00%)	0 / 5 (0.00%)	0 / 10 (0.00%)
occurrences (all)	0	0	0
Psychiatric disorders			
Anxiety			
subjects affected / exposed	1 / 14 (7.14%)	0 / 5 (0.00%)	1 / 10 (10.00%)
occurrences (all)	2	0	1
Attention deficit/hyperactivity disorder			

subjects affected / exposed	0 / 14 (0.00%)	0 / 5 (0.00%)	0 / 10 (0.00%)
occurrences (all)	0	0	0
Mood altered			
subjects affected / exposed	0 / 14 (0.00%)	0 / 5 (0.00%)	1 / 10 (10.00%)
occurrences (all)	0	0	2
Restlessness			
subjects affected / exposed	0 / 14 (0.00%)	0 / 5 (0.00%)	0 / 10 (0.00%)
occurrences (all)	0	0	0
Sleep disorder			
subjects affected / exposed	0 / 14 (0.00%)	0 / 5 (0.00%)	1 / 10 (10.00%)
occurrences (all)	0	0	1
Investigations			
Blood bilirubin increased			
subjects affected / exposed	0 / 14 (0.00%)	0 / 5 (0.00%)	1 / 10 (10.00%)
occurrences (all)	0	0	1
Blood fibrinogen increased			
subjects affected / exposed	0 / 14 (0.00%)	0 / 5 (0.00%)	0 / 10 (0.00%)
occurrences (all)	0	0	0
Blood lactic acid increased			
subjects affected / exposed	0 / 14 (0.00%)	0 / 5 (0.00%)	0 / 10 (0.00%)
occurrences (all)	0	0	0
Blood pressure increased			
subjects affected / exposed	0 / 14 (0.00%)	0 / 5 (0.00%)	1 / 10 (10.00%)
occurrences (all)	0	0	3
Body temperature increased			
subjects affected / exposed	0 / 14 (0.00%)	0 / 5 (0.00%)	1 / 10 (10.00%)
occurrences (all)	0	0	1
C-reactive protein increased			
subjects affected / exposed	0 / 14 (0.00%)	0 / 5 (0.00%)	0 / 10 (0.00%)
occurrences (all)	0	0	0
Complement factor C3 increased			
subjects affected / exposed	0 / 14 (0.00%)	0 / 5 (0.00%)	0 / 10 (0.00%)
occurrences (all)	0	0	0
Ejection fraction decreased			
subjects affected / exposed	1 / 14 (7.14%)	0 / 5 (0.00%)	0 / 10 (0.00%)
occurrences (all)	1	0	0

Gamma-glutamyltransferase increased			
subjects affected / exposed	0 / 14 (0.00%)	0 / 5 (0.00%)	1 / 10 (10.00%)
occurrences (all)	0	0	1
Glutamate dehydrogenase increased			
subjects affected / exposed	0 / 14 (0.00%)	0 / 5 (0.00%)	2 / 10 (20.00%)
occurrences (all)	0	0	3
Heart rate increased			
subjects affected / exposed	0 / 14 (0.00%)	0 / 5 (0.00%)	1 / 10 (10.00%)
occurrences (all)	0	0	1
Monocyte count increased			
subjects affected / exposed	0 / 14 (0.00%)	0 / 5 (0.00%)	0 / 10 (0.00%)
occurrences (all)	0	0	0
Neutrophil count increased			
subjects affected / exposed	0 / 14 (0.00%)	0 / 5 (0.00%)	0 / 10 (0.00%)
occurrences (all)	0	0	0
Protein urine present			
subjects affected / exposed	0 / 14 (0.00%)	0 / 5 (0.00%)	1 / 10 (10.00%)
occurrences (all)	0	0	1
Troponin I increased			
subjects affected / exposed	0 / 14 (0.00%)	0 / 5 (0.00%)	1 / 10 (10.00%)
occurrences (all)	0	0	1
Troponin increased			
subjects affected / exposed	1 / 14 (7.14%)	0 / 5 (0.00%)	0 / 10 (0.00%)
occurrences (all)	1	0	0
Weight decreased			
subjects affected / exposed	0 / 14 (0.00%)	0 / 5 (0.00%)	1 / 10 (10.00%)
occurrences (all)	0	0	1
White blood cell count increased			
subjects affected / exposed	0 / 14 (0.00%)	0 / 5 (0.00%)	0 / 10 (0.00%)
occurrences (all)	0	0	0
Injury, poisoning and procedural complications			
Arthropod bite			
subjects affected / exposed	0 / 14 (0.00%)	0 / 5 (0.00%)	0 / 10 (0.00%)
occurrences (all)	0	0	0
Arthropod sting			

subjects affected / exposed	0 / 14 (0.00%)	0 / 5 (0.00%)	1 / 10 (10.00%)
occurrences (all)	0	0	1
Fall			
subjects affected / exposed	1 / 14 (7.14%)	0 / 5 (0.00%)	2 / 10 (20.00%)
occurrences (all)	1	0	2
Heat exhaustion			
subjects affected / exposed	0 / 14 (0.00%)	0 / 5 (0.00%)	1 / 10 (10.00%)
occurrences (all)	0	0	1
Laceration			
subjects affected / exposed	0 / 14 (0.00%)	0 / 5 (0.00%)	0 / 10 (0.00%)
occurrences (all)	0	0	0
Ligament sprain			
subjects affected / exposed	2 / 14 (14.29%)	0 / 5 (0.00%)	1 / 10 (10.00%)
occurrences (all)	2	0	1
Limb injury			
subjects affected / exposed	0 / 14 (0.00%)	0 / 5 (0.00%)	1 / 10 (10.00%)
occurrences (all)	0	0	1
Procedural pain			
subjects affected / exposed	0 / 14 (0.00%)	0 / 5 (0.00%)	2 / 10 (20.00%)
occurrences (all)	0	0	2
Skin abrasion			
subjects affected / exposed	0 / 14 (0.00%)	0 / 5 (0.00%)	0 / 10 (0.00%)
occurrences (all)	0	0	0
Soft tissue injury			
subjects affected / exposed	0 / 14 (0.00%)	0 / 5 (0.00%)	0 / 10 (0.00%)
occurrences (all)	0	0	0
Wound complication			
subjects affected / exposed	0 / 14 (0.00%)	0 / 5 (0.00%)	1 / 10 (10.00%)
occurrences (all)	0	0	1
Wound secretion			
subjects affected / exposed	0 / 14 (0.00%)	0 / 5 (0.00%)	0 / 10 (0.00%)
occurrences (all)	0	0	0
Cardiac disorders			
Cyanosis			
subjects affected / exposed	0 / 14 (0.00%)	0 / 5 (0.00%)	0 / 10 (0.00%)
occurrences (all)	0	0	0

Palpitations			
subjects affected / exposed	0 / 14 (0.00%)	0 / 5 (0.00%)	0 / 10 (0.00%)
occurrences (all)	0	0	0
Sinus tachycardia			
subjects affected / exposed	0 / 14 (0.00%)	0 / 5 (0.00%)	1 / 10 (10.00%)
occurrences (all)	0	0	2
Tachycardia			
subjects affected / exposed	0 / 14 (0.00%)	0 / 5 (0.00%)	0 / 10 (0.00%)
occurrences (all)	0	0	0
Nervous system disorders			
Dizziness			
subjects affected / exposed	0 / 14 (0.00%)	0 / 5 (0.00%)	2 / 10 (20.00%)
occurrences (all)	0	0	2
Dysstasia			
subjects affected / exposed	1 / 14 (7.14%)	0 / 5 (0.00%)	0 / 10 (0.00%)
occurrences (all)	1	0	0
Headache			
subjects affected / exposed	2 / 14 (14.29%)	1 / 5 (20.00%)	6 / 10 (60.00%)
occurrences (all)	4	1	37
Lethargy			
subjects affected / exposed	0 / 14 (0.00%)	0 / 5 (0.00%)	1 / 10 (10.00%)
occurrences (all)	0	0	1
Ear and labyrinth disorders			
Ear pain			
subjects affected / exposed	0 / 14 (0.00%)	0 / 5 (0.00%)	1 / 10 (10.00%)
occurrences (all)	0	0	1
Tympanic membrane hyperaemia			
subjects affected / exposed	0 / 14 (0.00%)	0 / 5 (0.00%)	1 / 10 (10.00%)
occurrences (all)	0	0	1
Eye disorders			
Eye pruritus			
subjects affected / exposed	0 / 14 (0.00%)	0 / 5 (0.00%)	0 / 10 (0.00%)
occurrences (all)	0	0	0
Gastrointestinal disorders			
Abdominal discomfort			
subjects affected / exposed	0 / 14 (0.00%)	0 / 5 (0.00%)	1 / 10 (10.00%)
occurrences (all)	0	0	1

Abdominal pain			
subjects affected / exposed	1 / 14 (7.14%)	1 / 5 (20.00%)	2 / 10 (20.00%)
occurrences (all)	2	1	6
Abdominal pain upper			
subjects affected / exposed	1 / 14 (7.14%)	0 / 5 (0.00%)	4 / 10 (40.00%)
occurrences (all)	1	0	4
Defaecation urgency			
subjects affected / exposed	0 / 14 (0.00%)	0 / 5 (0.00%)	1 / 10 (10.00%)
occurrences (all)	0	0	1
Diarrhoea			
subjects affected / exposed	0 / 14 (0.00%)	0 / 5 (0.00%)	3 / 10 (30.00%)
occurrences (all)	0	0	9
Dysphagia			
subjects affected / exposed	0 / 14 (0.00%)	0 / 5 (0.00%)	0 / 10 (0.00%)
occurrences (all)	0	0	0
Flatulence			
subjects affected / exposed	0 / 14 (0.00%)	0 / 5 (0.00%)	0 / 10 (0.00%)
occurrences (all)	0	0	0
Food poisoning			
subjects affected / exposed	0 / 14 (0.00%)	0 / 5 (0.00%)	0 / 10 (0.00%)
occurrences (all)	0	0	0
Gastritis			
subjects affected / exposed	0 / 14 (0.00%)	0 / 5 (0.00%)	0 / 10 (0.00%)
occurrences (all)	0	0	0
Haemorrhoids			
subjects affected / exposed	0 / 14 (0.00%)	0 / 5 (0.00%)	1 / 10 (10.00%)
occurrences (all)	0	0	1
Loose tooth			
subjects affected / exposed	0 / 14 (0.00%)	0 / 5 (0.00%)	1 / 10 (10.00%)
occurrences (all)	0	0	2
Nausea			
subjects affected / exposed	1 / 14 (7.14%)	0 / 5 (0.00%)	1 / 10 (10.00%)
occurrences (all)	1	0	2
Stomatitis			
subjects affected / exposed	0 / 14 (0.00%)	0 / 5 (0.00%)	0 / 10 (0.00%)
occurrences (all)	0	0	0

Vomiting			
subjects affected / exposed	1 / 14 (7.14%)	0 / 5 (0.00%)	3 / 10 (30.00%)
occurrences (all)	1	0	3
Skin and subcutaneous tissue disorders			
Dermatitis contact			
subjects affected / exposed	0 / 14 (0.00%)	0 / 5 (0.00%)	1 / 10 (10.00%)
occurrences (all)	0	0	1
Eczema			
subjects affected / exposed	0 / 14 (0.00%)	1 / 5 (20.00%)	0 / 10 (0.00%)
occurrences (all)	0	1	0
Erythema			
subjects affected / exposed	1 / 14 (7.14%)	2 / 5 (40.00%)	0 / 10 (0.00%)
occurrences (all)	1	2	0
Livedo reticularis			
subjects affected / exposed	0 / 14 (0.00%)	0 / 5 (0.00%)	0 / 10 (0.00%)
occurrences (all)	0	0	0
Pruritus			
subjects affected / exposed	1 / 14 (7.14%)	0 / 5 (0.00%)	0 / 10 (0.00%)
occurrences (all)	1	0	0
Rash			
subjects affected / exposed	0 / 14 (0.00%)	1 / 5 (20.00%)	2 / 10 (20.00%)
occurrences (all)	0	1	2
Rash erythematous			
subjects affected / exposed	0 / 14 (0.00%)	1 / 5 (20.00%)	0 / 10 (0.00%)
occurrences (all)	0	1	0
Rash maculo-papular			
subjects affected / exposed	0 / 14 (0.00%)	0 / 5 (0.00%)	0 / 10 (0.00%)
occurrences (all)	0	0	0
Rash pruritic			
subjects affected / exposed	0 / 14 (0.00%)	0 / 5 (0.00%)	0 / 10 (0.00%)
occurrences (all)	0	0	0
Urticaria			
subjects affected / exposed	0 / 14 (0.00%)	0 / 5 (0.00%)	1 / 10 (10.00%)
occurrences (all)	0	0	1
Renal and urinary disorders			

Dysuria			
subjects affected / exposed	0 / 14 (0.00%)	0 / 5 (0.00%)	0 / 10 (0.00%)
occurrences (all)	0	0	0
Musculoskeletal and connective tissue disorders			
Arthralgia			
subjects affected / exposed	1 / 14 (7.14%)	1 / 5 (20.00%)	0 / 10 (0.00%)
occurrences (all)	1	3	0
Back pain			
subjects affected / exposed	0 / 14 (0.00%)	0 / 5 (0.00%)	2 / 10 (20.00%)
occurrences (all)	0	0	3
Haemarthrosis			
subjects affected / exposed	0 / 14 (0.00%)	0 / 5 (0.00%)	0 / 10 (0.00%)
occurrences (all)	0	0	0
Musculoskeletal chest pain			
subjects affected / exposed	0 / 14 (0.00%)	1 / 5 (20.00%)	0 / 10 (0.00%)
occurrences (all)	0	1	0
Musculoskeletal pain			
subjects affected / exposed	0 / 14 (0.00%)	0 / 5 (0.00%)	1 / 10 (10.00%)
occurrences (all)	0	0	1
Myalgia			
subjects affected / exposed	1 / 14 (7.14%)	0 / 5 (0.00%)	0 / 10 (0.00%)
occurrences (all)	1	0	0
Osteoporosis			
subjects affected / exposed	0 / 14 (0.00%)	0 / 5 (0.00%)	0 / 10 (0.00%)
occurrences (all)	0	0	0
Pain in extremity			
subjects affected / exposed	1 / 14 (7.14%)	0 / 5 (0.00%)	1 / 10 (10.00%)
occurrences (all)	1	0	1
Infections and infestations			
Asymptomatic bacteriuria			
subjects affected / exposed	0 / 14 (0.00%)	0 / 5 (0.00%)	1 / 10 (10.00%)
occurrences (all)	0	0	1
Bronchitis			
subjects affected / exposed	1 / 14 (7.14%)	0 / 5 (0.00%)	0 / 10 (0.00%)
occurrences (all)	1	0	0
Candida infection			

subjects affected / exposed	0 / 14 (0.00%)	0 / 5 (0.00%)	0 / 10 (0.00%)
occurrences (all)	0	0	0
Cystitis			
subjects affected / exposed	0 / 14 (0.00%)	0 / 5 (0.00%)	0 / 10 (0.00%)
occurrences (all)	0	0	0
Gastroenteritis			
subjects affected / exposed	1 / 14 (7.14%)	0 / 5 (0.00%)	0 / 10 (0.00%)
occurrences (all)	1	0	0
Influenza			
subjects affected / exposed	1 / 14 (7.14%)	0 / 5 (0.00%)	0 / 10 (0.00%)
occurrences (all)	1	0	0
Nasopharyngitis			
subjects affected / exposed	1 / 14 (7.14%)	0 / 5 (0.00%)	2 / 10 (20.00%)
occurrences (all)	1	0	4
Rhinitis			
subjects affected / exposed	0 / 14 (0.00%)	1 / 5 (20.00%)	0 / 10 (0.00%)
occurrences (all)	0	1	0
Skin bacterial infection			
subjects affected / exposed	0 / 14 (0.00%)	0 / 5 (0.00%)	0 / 10 (0.00%)
occurrences (all)	0	0	0
Tinea infection			
subjects affected / exposed	1 / 14 (7.14%)	0 / 5 (0.00%)	0 / 10 (0.00%)
occurrences (all)	1	0	0
Upper respiratory tract infection			
subjects affected / exposed	0 / 14 (0.00%)	0 / 5 (0.00%)	0 / 10 (0.00%)
occurrences (all)	0	0	0
Wound infection			
subjects affected / exposed	0 / 14 (0.00%)	0 / 5 (0.00%)	1 / 10 (10.00%)
occurrences (all)	0	0	1
Metabolism and nutrition disorders			
Decreased appetite			
subjects affected / exposed	0 / 14 (0.00%)	0 / 5 (0.00%)	1 / 10 (10.00%)
occurrences (all)	0	0	1
Dehydration			
subjects affected / exposed	0 / 14 (0.00%)	0 / 5 (0.00%)	0 / 10 (0.00%)
occurrences (all)	0	0	0

Insulin resistance			
subjects affected / exposed	1 / 14 (7.14%)	0 / 5 (0.00%)	0 / 10 (0.00%)
occurrences (all)	1	0	0

Non-serious adverse events	5 mg/kg WVE-210201		
Total subjects affected by non-serious adverse events			
subjects affected / exposed	26 / 26 (100.00%)		
Vascular disorders			
Flushing			
subjects affected / exposed	3 / 26 (11.54%)		
occurrences (all)	13		
Haematoma			
subjects affected / exposed	0 / 26 (0.00%)		
occurrences (all)	0		
Hypertension			
subjects affected / exposed	1 / 26 (3.85%)		
occurrences (all)	1		
Pallor			
subjects affected / exposed	1 / 26 (3.85%)		
occurrences (all)	1		
General disorders and administration site conditions			
Catheter site erythema			
subjects affected / exposed	0 / 26 (0.00%)		
occurrences (all)	0		
Chest discomfort			
subjects affected / exposed	1 / 26 (3.85%)		
occurrences (all)	5		
Chest pain			
subjects affected / exposed	3 / 26 (11.54%)		
occurrences (all)	9		
Chills			
subjects affected / exposed	4 / 26 (15.38%)		
occurrences (all)	8		
Fatigue			
subjects affected / exposed	2 / 26 (7.69%)		
occurrences (all)	4		

Feeling abnormal			
subjects affected / exposed	1 / 26 (3.85%)		
occurrences (all)	1		
Feeling cold			
subjects affected / exposed	1 / 26 (3.85%)		
occurrences (all)	1		
Hyperthermia			
subjects affected / exposed	0 / 26 (0.00%)		
occurrences (all)	0		
Infusion site erythema			
subjects affected / exposed	1 / 26 (3.85%)		
occurrences (all)	1		
Infusion site papule			
subjects affected / exposed	1 / 26 (3.85%)		
occurrences (all)	2		
Malaise			
subjects affected / exposed	1 / 26 (3.85%)		
occurrences (all)	1		
Non-cardiac chest pain			
subjects affected / exposed	1 / 26 (3.85%)		
occurrences (all)	1		
Pain			
subjects affected / exposed	1 / 26 (3.85%)		
occurrences (all)	1		
Peripheral swelling			
subjects affected / exposed	1 / 26 (3.85%)		
occurrences (all)	1		
Pyrexia			
subjects affected / exposed	22 / 26 (84.62%)		
occurrences (all)	113		
Immune system disorders			
Allergy to arthropod bite			
subjects affected / exposed	0 / 26 (0.00%)		
occurrences (all)	0		
Seasonal allergy			

subjects affected / exposed	0 / 26 (0.00%)		
occurrences (all)	0		
Reproductive system and breast disorders			
Pelvic pain			
subjects affected / exposed	1 / 26 (3.85%)		
occurrences (all)	1		
Respiratory, thoracic and mediastinal disorders			
Cough			
subjects affected / exposed	5 / 26 (19.23%)		
occurrences (all)	7		
Dyspnoea			
subjects affected / exposed	1 / 26 (3.85%)		
occurrences (all)	4		
Epistaxis			
subjects affected / exposed	0 / 26 (0.00%)		
occurrences (all)	0		
Hypoxia			
subjects affected / exposed	1 / 26 (3.85%)		
occurrences (all)	1		
Nasal congestion			
subjects affected / exposed	3 / 26 (11.54%)		
occurrences (all)	3		
Oropharyngeal pain			
subjects affected / exposed	1 / 26 (3.85%)		
occurrences (all)	1		
Rhinorrhoea			
subjects affected / exposed	4 / 26 (15.38%)		
occurrences (all)	5		
Sneezing			
subjects affected / exposed	0 / 26 (0.00%)		
occurrences (all)	0		
Sputum discoloured			
subjects affected / exposed	0 / 26 (0.00%)		
occurrences (all)	0		
Throat irritation			

subjects affected / exposed	1 / 26 (3.85%)		
occurrences (all)	1		
Psychiatric disorders			
Anxiety			
subjects affected / exposed	1 / 26 (3.85%)		
occurrences (all)	1		
Attention deficit/hyperactivity disorder			
subjects affected / exposed	1 / 26 (3.85%)		
occurrences (all)	1		
Mood altered			
subjects affected / exposed	0 / 26 (0.00%)		
occurrences (all)	0		
Restlessness			
subjects affected / exposed	1 / 26 (3.85%)		
occurrences (all)	1		
Sleep disorder			
subjects affected / exposed	0 / 26 (0.00%)		
occurrences (all)	0		
Investigations			
Blood bilirubin increased			
subjects affected / exposed	0 / 26 (0.00%)		
occurrences (all)	0		
Blood fibrinogen increased			
subjects affected / exposed	1 / 26 (3.85%)		
occurrences (all)	1		
Blood lactic acid increased			
subjects affected / exposed	1 / 26 (3.85%)		
occurrences (all)	1		
Blood pressure increased			
subjects affected / exposed	0 / 26 (0.00%)		
occurrences (all)	0		
Body temperature increased			
subjects affected / exposed	2 / 26 (7.69%)		
occurrences (all)	3		
C-reactive protein increased			

subjects affected / exposed	1 / 26 (3.85%)			
occurrences (all)	1			
Complement factor C3 increased				
subjects affected / exposed	2 / 26 (7.69%)			
occurrences (all)	2			
Ejection fraction decreased				
subjects affected / exposed	0 / 26 (0.00%)			
occurrences (all)	0			
Gamma-glutamyltransferase increased				
subjects affected / exposed	2 / 26 (7.69%)			
occurrences (all)	2			
Glutamate dehydrogenase increased				
subjects affected / exposed	4 / 26 (15.38%)			
occurrences (all)	4			
Heart rate increased				
subjects affected / exposed	1 / 26 (3.85%)			
occurrences (all)	1			
Monocyte count increased				
subjects affected / exposed	1 / 26 (3.85%)			
occurrences (all)	1			
Neutrophil count increased				
subjects affected / exposed	1 / 26 (3.85%)			
occurrences (all)	1			
Protein urine present				
subjects affected / exposed	1 / 26 (3.85%)			
occurrences (all)	4			
Troponin I increased				
subjects affected / exposed	0 / 26 (0.00%)			
occurrences (all)	0			
Troponin increased				
subjects affected / exposed	0 / 26 (0.00%)			
occurrences (all)	0			
Weight decreased				
subjects affected / exposed	0 / 26 (0.00%)			
occurrences (all)	0			

White blood cell count increased			
subjects affected / exposed	1 / 26 (3.85%)		
occurrences (all)	1		
Injury, poisoning and procedural complications			
Arthropod bite			
subjects affected / exposed	2 / 26 (7.69%)		
occurrences (all)	2		
Arthropod sting			
subjects affected / exposed	0 / 26 (0.00%)		
occurrences (all)	0		
Fall			
subjects affected / exposed	9 / 26 (34.62%)		
occurrences (all)	14		
Heat exhaustion			
subjects affected / exposed	0 / 26 (0.00%)		
occurrences (all)	0		
Laceration			
subjects affected / exposed	1 / 26 (3.85%)		
occurrences (all)	1		
Ligament sprain			
subjects affected / exposed	0 / 26 (0.00%)		
occurrences (all)	0		
Limb injury			
subjects affected / exposed	2 / 26 (7.69%)		
occurrences (all)	2		
Procedural pain			
subjects affected / exposed	6 / 26 (23.08%)		
occurrences (all)	6		
Skin abrasion			
subjects affected / exposed	1 / 26 (3.85%)		
occurrences (all)	1		
Soft tissue injury			
subjects affected / exposed	1 / 26 (3.85%)		
occurrences (all)	1		
Wound complication			

subjects affected / exposed	0 / 26 (0.00%)		
occurrences (all)	0		
Wound secretion			
subjects affected / exposed	1 / 26 (3.85%)		
occurrences (all)	1		
Cardiac disorders			
Cyanosis			
subjects affected / exposed	1 / 26 (3.85%)		
occurrences (all)	1		
Palpitations			
subjects affected / exposed	1 / 26 (3.85%)		
occurrences (all)	1		
Sinus tachycardia			
subjects affected / exposed	0 / 26 (0.00%)		
occurrences (all)	0		
Tachycardia			
subjects affected / exposed	7 / 26 (26.92%)		
occurrences (all)	31		
Nervous system disorders			
Dizziness			
subjects affected / exposed	0 / 26 (0.00%)		
occurrences (all)	0		
Dysstasia			
subjects affected / exposed	0 / 26 (0.00%)		
occurrences (all)	0		
Headache			
subjects affected / exposed	14 / 26 (53.85%)		
occurrences (all)	58		
Lethargy			
subjects affected / exposed	1 / 26 (3.85%)		
occurrences (all)	1		
Ear and labyrinth disorders			
Ear pain			
subjects affected / exposed	0 / 26 (0.00%)		
occurrences (all)	0		
Tympanic membrane hyperaemia			

subjects affected / exposed	0 / 26 (0.00%)		
occurrences (all)	0		
Eye disorders			
Eye pruritus			
subjects affected / exposed	1 / 26 (3.85%)		
occurrences (all)	1		
Gastrointestinal disorders			
Abdominal discomfort			
subjects affected / exposed	0 / 26 (0.00%)		
occurrences (all)	0		
Abdominal pain			
subjects affected / exposed	6 / 26 (23.08%)		
occurrences (all)	14		
Abdominal pain upper			
subjects affected / exposed	4 / 26 (15.38%)		
occurrences (all)	7		
Defaecation urgency			
subjects affected / exposed	0 / 26 (0.00%)		
occurrences (all)	0		
Diarrhoea			
subjects affected / exposed	8 / 26 (30.77%)		
occurrences (all)	8		
Dysphagia			
subjects affected / exposed	1 / 26 (3.85%)		
occurrences (all)	1		
Flatulence			
subjects affected / exposed	1 / 26 (3.85%)		
occurrences (all)	1		
Food poisoning			
subjects affected / exposed	1 / 26 (3.85%)		
occurrences (all)	1		
Gastritis			
subjects affected / exposed	1 / 26 (3.85%)		
occurrences (all)	1		
Haemorrhoids			

subjects affected / exposed	0 / 26 (0.00%)		
occurrences (all)	0		
Loose tooth			
subjects affected / exposed	0 / 26 (0.00%)		
occurrences (all)	0		
Nausea			
subjects affected / exposed	8 / 26 (30.77%)		
occurrences (all)	17		
Stomatitis			
subjects affected / exposed	1 / 26 (3.85%)		
occurrences (all)	1		
Vomiting			
subjects affected / exposed	14 / 26 (53.85%)		
occurrences (all)	46		
Skin and subcutaneous tissue disorders			
Dermatitis contact			
subjects affected / exposed	0 / 26 (0.00%)		
occurrences (all)	0		
Eczema			
subjects affected / exposed	1 / 26 (3.85%)		
occurrences (all)	1		
Erythema			
subjects affected / exposed	0 / 26 (0.00%)		
occurrences (all)	0		
Livedo reticularis			
subjects affected / exposed	2 / 26 (7.69%)		
occurrences (all)	2		
Pruritus			
subjects affected / exposed	1 / 26 (3.85%)		
occurrences (all)	1		
Rash			
subjects affected / exposed	2 / 26 (7.69%)		
occurrences (all)	5		
Rash erythematous			
subjects affected / exposed	0 / 26 (0.00%)		
occurrences (all)	0		

Rash maculo-papular			
subjects affected / exposed	1 / 26 (3.85%)		
occurrences (all)	1		
Rash pruritic			
subjects affected / exposed	1 / 26 (3.85%)		
occurrences (all)	9		
Urticaria			
subjects affected / exposed	1 / 26 (3.85%)		
occurrences (all)	1		
Renal and urinary disorders			
Dysuria			
subjects affected / exposed	1 / 26 (3.85%)		
occurrences (all)	1		
Musculoskeletal and connective tissue disorders			
Arthralgia			
subjects affected / exposed	0 / 26 (0.00%)		
occurrences (all)	0		
Back pain			
subjects affected / exposed	5 / 26 (19.23%)		
occurrences (all)	5		
Haemarthrosis			
subjects affected / exposed	1 / 26 (3.85%)		
occurrences (all)	1		
Musculoskeletal chest pain			
subjects affected / exposed	1 / 26 (3.85%)		
occurrences (all)	7		
Musculoskeletal pain			
subjects affected / exposed	0 / 26 (0.00%)		
occurrences (all)	0		
Myalgia			
subjects affected / exposed	0 / 26 (0.00%)		
occurrences (all)	0		
Osteoporosis			
subjects affected / exposed	1 / 26 (3.85%)		
occurrences (all)	1		
Pain in extremity			

subjects affected / exposed	6 / 26 (23.08%)		
occurrences (all)	11		
Infections and infestations			
Asymptomatic bacteriuria			
subjects affected / exposed	0 / 26 (0.00%)		
occurrences (all)	0		
Bronchitis			
subjects affected / exposed	2 / 26 (7.69%)		
occurrences (all)	2		
Candida infection			
subjects affected / exposed	1 / 26 (3.85%)		
occurrences (all)	1		
Cystitis			
subjects affected / exposed	1 / 26 (3.85%)		
occurrences (all)	1		
Gastroenteritis			
subjects affected / exposed	2 / 26 (7.69%)		
occurrences (all)	4		
Influenza			
subjects affected / exposed	0 / 26 (0.00%)		
occurrences (all)	0		
Nasopharyngitis			
subjects affected / exposed	2 / 26 (7.69%)		
occurrences (all)	2		
Rhinitis			
subjects affected / exposed	0 / 26 (0.00%)		
occurrences (all)	0		
Skin bacterial infection			
subjects affected / exposed	1 / 26 (3.85%)		
occurrences (all)	1		
Tinea infection			
subjects affected / exposed	0 / 26 (0.00%)		
occurrences (all)	0		
Upper respiratory tract infection			
subjects affected / exposed	3 / 26 (11.54%)		
occurrences (all)	3		

Wound infection subjects affected / exposed occurrences (all)	0 / 26 (0.00%) 0		
Metabolism and nutrition disorders Decreased appetite subjects affected / exposed occurrences (all) Dehydration subjects affected / exposed occurrences (all) Insulin resistance subjects affected / exposed occurrences (all)	 2 / 26 (7.69%) 6 1 / 26 (3.85%) 1 0 / 26 (0.00%) 0		

More information

Substantial protocol amendments (globally)

Were there any global substantial amendments to the protocol? Yes

Date	Amendment
25 May 2018	Amendment 3.0, dated 25 May 2018 - Addition of liquid formulation of WVE-210201
01 August 2018	Amendment 5.0, dated 01 August 2018 - Baseline needle biopsy collection was removed and end-of-treatment needle biopsy was clarified.
22 January 2019	Amendment 6.0, dated 22 January 2019 - Study was extended from 14 weeks to 96 weeks. Dose modification to the highest tolerated doses (3.5 and 5 mg/kg) was added.

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.

Not applicable

Notes: