



## Clinical trial results:

### A Multicenter, Double-blind, Placebo-controlled, Phase 1 Study of WVE-210201 Administered Intravenously to Patients with Duchenne Muscular Dystrophy

#### Summary

EudraCT number	2017-002686-21
Trial protocol	GB FR BE NL IT
Global end of trial date	06 March 2019

#### Results information

Result version number	v1 (current)
This version publication date	26 March 2020
First version publication date	26 March 2020

#### Trial information

##### Trial identification

Sponsor protocol code	WVE-DMDX51-001
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##### Additional study identifiers

ISRCTN number	-
ClinicalTrials.gov id (NCT number)	NCT03508947
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, +44 (617) 949-2900, info@wavelifesci.com
Scientific contact	Chief Medical Officer, Wave Life Sciences, +44 (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	27 March 2019
Is this the analysis of the primary completion data?	Yes
Primary completion date	06 March 2019
Global end of trial reached?	Yes
Global end of trial date	06 March 2019
Was the trial ended prematurely?	No

Notes:

## General information about the trial

Main objective of the trial:

To evaluate the safety and tolerability of single ascending doses of WVE-210201 in patients with DM1.

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 will be 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 decision regarding escalation to each subsequent dose level was made based on the recommendation of the Dose Escalation Committee (DEC). The DEC was blinded to treatment assignment throughout the study.

The unblinded, independent Safety Monitoring Committee (SMC) reviewed aggregate safety data periodically and SAEs as they occurred. The SMC also reviewed any serious adverse events (SAEs) that occurred in sentinel patients in order to determine if the cohort should continue or, in conjunction with the DEC, whether an intermediate dose should be selected for the remaining patients in that cohort. In addition, if treatment-emergent adverse events (TEAEs) occurred that met the Dose Escalation Stopping Criteria, the SMC reviewed the unblinded safety data and determined whether it was safe to proceed with dosing.

Background therapy: -

Evidence for comparator: -

Actual start date of recruitment	24 January 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	Netherlands: 1
Country: Number of subjects enrolled	United Kingdom: 6
Country: Number of subjects enrolled	Belgium: 10
Country: Number of subjects enrolled	France: 5
Country: Number of subjects enrolled	Italy: 4
Country: Number of subjects enrolled	United States: 10
Worldwide total number of subjects	36
EEA total number of subjects	26

Notes:

<b>Subjects enrolled per age group</b>	
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)	34
Adolescents (12-17 years)	2
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, Netherlands, United Kingdom, and United States) from 24 January 2018 to 06 March 2019.

### Pre-assignment

Screening details:

A total of 42 subjects were screened and 36 subjects were enrolled and randomized to study treatment or placebo.

### Pre-assignment period milestones

Number of subjects started	36
Number of subjects completed	36

### Period 1

Period 1 title	Overall Study (overall period)
Is this the baseline period?	Yes
Allocation method	Randomised - controlled
Blinding used	Double blind
Roles blinded	Subject, Investigator, Data analyst, Carer, Assessor

### Arms

Are arms mutually exclusive?	Yes
<b>Arm title</b>	Pooled Placebo

Arm description:

0.9% Sodium Chloride injection or 0.45% Sodium Chloride injection solution administered alone via IV infusion

Arm type	Placebo Comparator
No investigational medicinal product assigned in this arm	
<b>Arm title</b>	0.5 mg/kg WVE-210201

Arm description:

0.5 mg/kg WVE-210201 administered via IV infusion

Arm type	Experimental
Investigational medicinal product name	Suvodirsén
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 a single IV infusion of suvodirsén or placebo.

<b>Arm title</b>	1 mg/kg WVE-210201
Arm description:	
1 mg/kg WVE-210201 administered via IV infusion	
Arm type	Experimental

Investigational medicinal product name	Suvodirsén
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 a single IV infusion of suvodirsén or placebo.

<b>Arm title</b>	2 mg/kg WVE-210201
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Arm description:

2 mg/kg WVE-210201 administered via IV infusion

Arm type	Experimental
Investigational medicinal product name	Suvodirsén
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 a single IV infusion of suvodirsén or placebo.

<b>Arm title</b>	5 mg/kg WVE-210201
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Arm description:

5 mg/kg WVE-210201 administered via IV infusion

Arm type	Experimental
Investigational medicinal product name	Suvodirsén
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 a single IV infusion of suvodirsén or placebo.

<b>Arm title</b>	7/10 mg/kg WVE-210201
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Arm description:

7 or 10 mg/kg administered via IV infusion

Arm type	Experimental
Investigational medicinal product name	Suvodirsén
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 a single IV infusion of suvodirsén or placebo.

<b>Number of subjects in period 1</b>	Pooled Placebo	0.5 mg/kg WVE-210201	1 mg/kg WVE-210201
Started	10	6	6
Completed	10	6	6

<b>Number of subjects in period 1</b>	2 mg/kg WVE- 210201	5 mg/kg WVE- 210201	7/10 mg/kg WVE- 210201
Started	6	6	2
Completed	6	6	2

## Baseline characteristics

### Reporting groups

Reporting group title	Pooled Placebo
Reporting group description: 0.9% Sodium Chloride injection or 0.45% Sodium Chloride injection solution administered alone via IV infusion	
Reporting group title	0.5 mg/kg WVE-210201
Reporting group description: 0.5 mg/kg WVE-210201 administered via IV infusion	
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	5 mg/kg WVE-210201
Reporting group description: 5 mg/kg WVE-210201 administered via IV infusion	
Reporting group title	7/10 mg/kg WVE-210201
Reporting group description: 7 or 10 mg/kg administered via IV infusion	

Reporting group values	Pooled Placebo	0.5 mg/kg WVE-210201	1 mg/kg WVE-210201
Number of subjects	10	6	6
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)	10	5	6
Adolescents (12-17 years)	0	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	10	6	6

Reporting group values	2 mg/kg WVE-210201	5 mg/kg WVE-210201	7/10 mg/kg WVE-210201
Number of subjects	6	6	2
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)	5	6	2
Adolescents (12-17 years)	1	0	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	6	6	2

Reporting group values	Total		
Number of subjects	36		
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)	34		
Adolescents (12-17 years)	2		
Adults (18-64 years)	0		
From 65-84 years	0		
85 years and over	0		
Gender categorical			
Units: Subjects			
Female	0		
Male	36		

### Subject analysis sets

Subject analysis set title	Safety population
Subject analysis set type	Safety analysis
Subject analysis set description:	
All randomly assigned patients who received at least 1 dose of WVE-210201 or placebo	

Reporting group values	Safety population		
Number of subjects	36		
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)	34		



Adolescents (12-17 years)	2		
Adults (18-64 years)	0		
From 65-84 years	0		
85 years and over	0		
Gender categorical			
Units: Subjects			
Female	0		
Male	36		

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## End points

### End points reporting groups

Reporting group title	Pooled Placebo
Reporting group description: 0.9% Sodium Chloride injection or 0.45% Sodium Chloride injection solution administered alone via IV infusion	
Reporting group title	0.5 mg/kg WVE-210201
Reporting group description: 0.5 mg/kg WVE-210201 administered via IV infusion	
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	5 mg/kg WVE-210201
Reporting group description: 5 mg/kg WVE-210201 administered via IV infusion	
Reporting group title	7/10 mg/kg WVE-210201
Reporting group description: 7 or 10 mg/kg administered via IV infusion	
Subject analysis set title	Safety population
Subject analysis set type	Safety analysis
Subject analysis set description: All randomly assigned patients who received at least 1 dose of WVE-210201 or placebo	

### Primary: Number of patients with treatment-emergent adverse events (TEAEs)

End point title	Number of patients with treatment-emergent adverse events (TEAEs) <sup>[1]</sup>
End point description: A TEAE was defined as any untoward medical occurrence in a patient following study drug administration, regardless of its causal relationship to study treatment.	
End point type	Primary
End point timeframe: Day 1 to Day 85 (end of study)	

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 were performed

End point values	Pooled Placebo	0.5 mg/kg WVE-210201	1 mg/kg WVE-210201	2 mg/kg WVE-210201
Subject group type	Reporting group	Reporting group	Reporting group	Reporting group
Number of subjects analysed	10	6	6	6
Units: Subjects				
number (not applicable)	8	3	5	4

End point values	5 mg/kg WVE-210201	7/10 mg/kg WVE-210201		
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Subject group type	Reporting group	Reporting group		
Number of subjects analysed	6	2		
Units: Subjects				
number (not applicable)	4	2		

## Statistical analyses

No statistical analyses for this end point

### Primary: Number of Patients who Experienced a Severe TEAE

End point title	Number of Patients who Experienced a Severe TEAE <sup>[2]</sup>
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End point description:

End point type	Primary
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End point timeframe:

Day 1 to Day 85 (end of study)

Notes:

[2] - 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 were performed

End point values	Pooled Placebo	0.5 mg/kg WVE-210201	1 mg/kg WVE-210201	2 mg/kg WVE-210201
Subject group type	Reporting group	Reporting group	Reporting group	Reporting group
Number of subjects analysed	10	6	6	6
Units: Subjects				
number (not applicable)				
Number of Patients who Experienced a Severe TEAE	2	0	0	0

End point values	5 mg/kg WVE-210201	7/10 mg/kg WVE-210201		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	6	2		
Units: Subjects				
number (not applicable)				
Number of Patients who Experienced a Severe TEAE	0	2		

## Statistical analyses

No statistical analyses for this end point

### Primary: Number of Patients who Experienced a Serious TEAE

End point title	Number of Patients who Experienced a Serious TEAE <sup>[3]</sup>
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**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.

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End point type	Primary
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**End point timeframe:**

Day 1 to Day 85 (end of study)

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**Notes:**

[3] - 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 were performed

<b>End point values</b>	Pooled Placebo	0.5 mg/kg WVE-210201	1 mg/kg WVE-210201	2 mg/kg WVE-210201
Subject group type	Reporting group	Reporting group	Reporting group	Reporting group
Number of subjects analysed	10	6	6	6
Units: Subjects				
number (not applicable)				
Number of Patients who Experienced an SAE	1	0	0	0

<b>End point values</b>	5 mg/kg WVE-210201	7/10 mg/kg WVE-210201		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	6	2		
Units: Subjects				
number (not applicable)				
Number of Patients who Experienced an SAE	0	0		

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**Statistical analyses**

No statistical analyses for this end point

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## Adverse events

### Adverse events information

Timeframe for reporting adverse events:

Day 1 to Day 85 (end of study)

Assessment type	Systematic
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### Dictionary used

Dictionary name	MedDRA
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Dictionary version	20.1
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### Reporting groups

Reporting group title	Pooled Placebo
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Reporting group description: -

Reporting group title	0.5 mg/kg
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Reporting group description: -

Reporting group title	1 mg/kg
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Reporting group description: -

Reporting group title	2 mg/kg
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Reporting group description: -

Reporting group title	5 mg/kg
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Reporting group description: -

Reporting group title	7/10 mg/kg
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Reporting group description: -

Serious adverse events	Pooled Placebo	0.5 mg/kg	1 mg/kg
Total subjects affected by serious adverse events			
subjects affected / exposed	1 / 10 (10.00%)	0 / 6 (0.00%)	0 / 6 (0.00%)
number of deaths (all causes)	0	0	0
number of deaths resulting from adverse events	0	0	0
Infections and infestations			
Corona virus infection			
subjects affected / exposed	1 / 10 (10.00%)	0 / 6 (0.00%)	0 / 6 (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
Influenza			
subjects affected / exposed	1 / 10 (10.00%)	0 / 6 (0.00%)	0 / 6 (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

Serious adverse events	2 mg/kg	5 mg/kg	7/10 mg/kg
Total subjects affected by serious adverse events			
subjects affected / exposed	0 / 6 (0.00%)	0 / 6 (0.00%)	0 / 2 (0.00%)

number of deaths (all causes)	0	0	0
number of deaths resulting from adverse events	0	0	0
Infections and infestations			
Corona virus infection			
subjects affected / exposed	0 / 6 (0.00%)	0 / 6 (0.00%)	0 / 2 (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
Influenza			
subjects affected / exposed	0 / 6 (0.00%)	0 / 6 (0.00%)	0 / 2 (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

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

<b>Non-serious adverse events</b>	Pooled Placebo	0.5 mg/kg	1 mg/kg
Total subjects affected by non-serious adverse events			
subjects affected / exposed	8 / 10 (80.00%)	5 / 6 (83.33%)	5 / 6 (83.33%)
General disorders and administration site conditions			
Catheter site pain			
subjects affected / exposed	1 / 10 (10.00%)	0 / 6 (0.00%)	0 / 6 (0.00%)
occurrences (all)	1	0	0
Chills			
subjects affected / exposed	0 / 10 (0.00%)	0 / 6 (0.00%)	0 / 6 (0.00%)
occurrences (all)	0	0	0
Fatigue			
subjects affected / exposed	0 / 10 (0.00%)	0 / 6 (0.00%)	0 / 6 (0.00%)
occurrences (all)	0	0	0
Influenza like illness			
subjects affected / exposed	1 / 10 (10.00%)	0 / 6 (0.00%)	0 / 6 (0.00%)
occurrences (all)	1	0	0
Infusion site pain			
subjects affected / exposed	0 / 10 (0.00%)	0 / 6 (0.00%)	0 / 6 (0.00%)
occurrences (all)	0	0	0
Peripheral swelling			

subjects affected / exposed occurrences (all)	0 / 10 (0.00%) 0	0 / 6 (0.00%) 0	0 / 6 (0.00%) 0
Pyrexia subjects affected / exposed occurrences (all)	0 / 10 (0.00%) 0	0 / 6 (0.00%) 0	0 / 6 (0.00%) 0
Respiratory, thoracic and mediastinal disorders Cough subjects affected / exposed occurrences (all)	0 / 10 (0.00%) 0	0 / 6 (0.00%) 0	1 / 6 (16.67%) 1
Epistaxis subjects affected / exposed occurrences (all)	1 / 10 (10.00%) 1	0 / 6 (0.00%) 0	0 / 6 (0.00%) 0
Nasal congestion subjects affected / exposed occurrences (all)	0 / 10 (0.00%) 0	1 / 6 (16.67%) 1	0 / 6 (0.00%) 0
Pharyngeal erythema subjects affected / exposed occurrences (all)	0 / 10 (0.00%) 0	0 / 6 (0.00%) 0	0 / 6 (0.00%) 0
Tonsillar hypertrophy subjects affected / exposed occurrences (all)	0 / 10 (0.00%) 0	0 / 6 (0.00%) 0	0 / 6 (0.00%) 0
Investigations Body temperature increased subjects affected / exposed occurrences (all)	1 / 10 (10.00%) 1	0 / 6 (0.00%) 0	0 / 6 (0.00%) 0
C-reactive protein increased subjects affected / exposed occurrences (all)	0 / 10 (0.00%) 0	0 / 6 (0.00%) 0	0 / 6 (0.00%) 0
Neutrophil count increased subjects affected / exposed occurrences (all)	0 / 10 (0.00%) 0	0 / 6 (0.00%) 0	1 / 6 (16.67%) 1
Injury, poisoning and procedural complications Fall subjects affected / exposed occurrences (all)	1 / 10 (10.00%) 1	1 / 6 (16.67%) 1	0 / 6 (0.00%) 0
Joint injury			

subjects affected / exposed occurrences (all)	0 / 10 (0.00%) 0	0 / 6 (0.00%) 0	0 / 6 (0.00%) 0
Cardiac disorders			
Bradycardia			
subjects affected / exposed	0 / 10 (0.00%)	0 / 6 (0.00%)	1 / 6 (16.67%)
occurrences (all)	0	0	1
Sinus bradycardia			
subjects affected / exposed	0 / 10 (0.00%)	0 / 6 (0.00%)	1 / 6 (16.67%)
occurrences (all)	0	0	1
Tachycardia			
subjects affected / exposed	1 / 10 (10.00%)	0 / 6 (0.00%)	0 / 6 (0.00%)
occurrences (all)	1	0	0
Nervous system disorders			
Headache			
subjects affected / exposed	0 / 10 (0.00%)	0 / 6 (0.00%)	2 / 6 (33.33%)
occurrences (all)	0	0	3
Tremor			
subjects affected / exposed	0 / 10 (0.00%)	0 / 6 (0.00%)	0 / 6 (0.00%)
occurrences (all)	0	0	0
Blood and lymphatic system disorders			
Leukocytosis			
subjects affected / exposed	0 / 10 (0.00%)	0 / 6 (0.00%)	0 / 6 (0.00%)
occurrences (all)	0	0	0
Ear and labyrinth disorders			
Ear pain			
subjects affected / exposed	0 / 10 (0.00%)	0 / 6 (0.00%)	0 / 6 (0.00%)
occurrences (all)	0	0	0
Tympanic membrane disorder			
subjects affected / exposed	0 / 10 (0.00%)	0 / 6 (0.00%)	0 / 6 (0.00%)
occurrences (all)	0	0	0
Tympanic membrane hyperaemia			
subjects affected / exposed	0 / 10 (0.00%)	0 / 6 (0.00%)	0 / 6 (0.00%)
occurrences (all)	0	0	0
Eye disorders			
Blepharospasm			
subjects affected / exposed	0 / 10 (0.00%)	0 / 6 (0.00%)	0 / 6 (0.00%)
occurrences (all)	0	0	0



Gastrointestinal disorders			
Abdominal pain			
subjects affected / exposed	0 / 10 (0.00%)	0 / 6 (0.00%)	0 / 6 (0.00%)
occurrences (all)	0	0	0
Abdominal pain upper			
subjects affected / exposed	0 / 10 (0.00%)	1 / 6 (16.67%)	0 / 6 (0.00%)
occurrences (all)	0	1	0
Abnormal faeces			
subjects affected / exposed	0 / 10 (0.00%)	0 / 6 (0.00%)	0 / 6 (0.00%)
occurrences (all)	0	0	0
Diarrhoea			
subjects affected / exposed	1 / 10 (10.00%)	0 / 6 (0.00%)	0 / 6 (0.00%)
occurrences (all)	1	0	0
Eructation			
subjects affected / exposed	0 / 10 (0.00%)	0 / 6 (0.00%)	0 / 6 (0.00%)
occurrences (all)	0	0	0
Gingival erythema			
subjects affected / exposed	1 / 10 (10.00%)	0 / 6 (0.00%)	0 / 6 (0.00%)
occurrences (all)	1	0	0
Gingival swelling			
subjects affected / exposed	1 / 10 (10.00%)	0 / 6 (0.00%)	0 / 6 (0.00%)
occurrences (all)	1	0	0
Nausea			
subjects affected / exposed	0 / 10 (0.00%)	1 / 6 (16.67%)	0 / 6 (0.00%)
occurrences (all)	0	1	0
Vomiting			
subjects affected / exposed	0 / 10 (0.00%)	1 / 6 (16.67%)	0 / 6 (0.00%)
occurrences (all)	0	1	0
Skin and subcutaneous tissue disorders			
Erythema			
subjects affected / exposed	0 / 10 (0.00%)	0 / 6 (0.00%)	1 / 6 (16.67%)
occurrences (all)	0	0	1
Hyperhidrosis			
subjects affected / exposed	0 / 10 (0.00%)	0 / 6 (0.00%)	0 / 6 (0.00%)
occurrences (all)	0	0	0
Livedo reticularis			

subjects affected / exposed occurrences (all)	0 / 10 (0.00%) 0	0 / 6 (0.00%) 0	0 / 6 (0.00%) 0
Pruritus subjects affected / exposed occurrences (all)	0 / 10 (0.00%) 0	0 / 6 (0.00%) 0	0 / 6 (0.00%) 0
Renal and urinary disorders Dysuria subjects affected / exposed occurrences (all)	0 / 10 (0.00%) 0	0 / 6 (0.00%) 0	1 / 6 (16.67%) 1
Musculoskeletal and connective tissue disorders Arthralgia subjects affected / exposed occurrences (all)	1 / 10 (10.00%) 1	0 / 6 (0.00%) 0	0 / 6 (0.00%) 0
Back pain subjects affected / exposed occurrences (all)	0 / 10 (0.00%) 0	0 / 6 (0.00%) 0	1 / 6 (16.67%) 1
Pain in extremity subjects affected / exposed occurrences (all)	2 / 10 (20.00%) 2	0 / 6 (0.00%) 0	0 / 6 (0.00%) 0
Infections and infestations Bullous impetigo subjects affected / exposed occurrences (all)	0 / 10 (0.00%) 0	1 / 6 (16.67%) 1	0 / 6 (0.00%) 0
Ear infection subjects affected / exposed occurrences (all)	0 / 10 (0.00%) 0	0 / 6 (0.00%) 0	1 / 6 (16.67%) 1
Gastroenteritis viral subjects affected / exposed occurrences (all)	0 / 10 (0.00%) 0	0 / 6 (0.00%) 0	0 / 6 (0.00%) 0
Nasopharyngitis subjects affected / exposed occurrences (all)	2 / 10 (20.00%) 3	0 / 6 (0.00%) 0	1 / 6 (16.67%) 1
Otitis media subjects affected / exposed occurrences (all)	1 / 10 (10.00%) 1	0 / 6 (0.00%) 0	0 / 6 (0.00%) 0
Tooth abscess			

subjects affected / exposed	1 / 10 (10.00%)	0 / 6 (0.00%)	0 / 6 (0.00%)
occurrences (all)	1	0	0
Upper respiratory tract infection			
subjects affected / exposed	1 / 10 (10.00%)	0 / 6 (0.00%)	0 / 6 (0.00%)
occurrences (all)	1	0	0
Viral upper respiratory tract infection			
subjects affected / exposed	0 / 10 (0.00%)	0 / 6 (0.00%)	0 / 6 (0.00%)
occurrences (all)	0	0	0
Metabolism and nutrition disorders			
Decreased appetite			
subjects affected / exposed	0 / 10 (0.00%)	0 / 6 (0.00%)	1 / 6 (16.67%)
occurrences (all)	0	0	1

<b>Non-serious adverse events</b>	2 mg/kg	5 mg/kg	7/10 mg/kg
Total subjects affected by non-serious adverse events			
subjects affected / exposed	4 / 6 (66.67%)	4 / 6 (66.67%)	2 / 2 (100.00%)
General disorders and administration site conditions			
Catheter site pain			
subjects affected / exposed	0 / 6 (0.00%)	0 / 6 (0.00%)	0 / 2 (0.00%)
occurrences (all)	0	0	0
Chills			
subjects affected / exposed	0 / 6 (0.00%)	0 / 6 (0.00%)	1 / 2 (50.00%)
occurrences (all)	0	0	1
Fatigue			
subjects affected / exposed	1 / 6 (16.67%)	0 / 6 (0.00%)	1 / 2 (50.00%)
occurrences (all)	1	0	1
Influenza like illness			
subjects affected / exposed	0 / 6 (0.00%)	0 / 6 (0.00%)	0 / 2 (0.00%)
occurrences (all)	0	0	0
Infusion site pain			
subjects affected / exposed	1 / 6 (16.67%)	0 / 6 (0.00%)	0 / 2 (0.00%)
occurrences (all)	1	0	0
Peripheral swelling			
subjects affected / exposed	1 / 6 (16.67%)	0 / 6 (0.00%)	0 / 2 (0.00%)
occurrences (all)	1	0	0
Pyrexia			

subjects affected / exposed occurrences (all)	0 / 6 (0.00%) 0	4 / 6 (66.67%) 4	2 / 2 (100.00%) 2
Respiratory, thoracic and mediastinal disorders			
Cough			
subjects affected / exposed	1 / 6 (16.67%)	1 / 6 (16.67%)	0 / 2 (0.00%)
occurrences (all)	2	1	0
Epistaxis			
subjects affected / exposed	0 / 6 (0.00%)	0 / 6 (0.00%)	0 / 2 (0.00%)
occurrences (all)	0	0	0
Nasal congestion			
subjects affected / exposed	0 / 6 (0.00%)	0 / 6 (0.00%)	0 / 2 (0.00%)
occurrences (all)	0	0	0
Pharyngeal erythema			
subjects affected / exposed	0 / 6 (0.00%)	1 / 6 (16.67%)	0 / 2 (0.00%)
occurrences (all)	0	1	0
Tonsillar hypertrophy			
subjects affected / exposed	0 / 6 (0.00%)	0 / 6 (0.00%)	1 / 2 (50.00%)
occurrences (all)	0	0	1
Investigations			
Body temperature increased			
subjects affected / exposed	0 / 6 (0.00%)	0 / 6 (0.00%)	0 / 2 (0.00%)
occurrences (all)	0	0	0
C-reactive protein increased			
subjects affected / exposed	0 / 6 (0.00%)	0 / 6 (0.00%)	1 / 2 (50.00%)
occurrences (all)	0	0	1
Neutrophil count increased			
subjects affected / exposed	0 / 6 (0.00%)	0 / 6 (0.00%)	1 / 2 (50.00%)
occurrences (all)	0	0	1
Injury, poisoning and procedural complications			
Fall			
subjects affected / exposed	1 / 6 (16.67%)	0 / 6 (0.00%)	0 / 2 (0.00%)
occurrences (all)	1	0	0
Joint injury			
subjects affected / exposed	0 / 6 (0.00%)	1 / 6 (16.67%)	0 / 2 (0.00%)
occurrences (all)	0	1	0
Cardiac disorders			

Bradycardia subjects affected / exposed occurrences (all)	0 / 6 (0.00%) 0	0 / 6 (0.00%) 0	0 / 2 (0.00%) 0
Sinus bradycardia subjects affected / exposed occurrences (all)	0 / 6 (0.00%) 0	0 / 6 (0.00%) 0	0 / 2 (0.00%) 0
Tachycardia subjects affected / exposed occurrences (all)	0 / 6 (0.00%) 0	1 / 6 (16.67%) 1	2 / 2 (100.00%) 2
Nervous system disorders Headache subjects affected / exposed occurrences (all)	1 / 6 (16.67%) 1	3 / 6 (50.00%) 3	1 / 2 (50.00%) 1
Tremor subjects affected / exposed occurrences (all)	0 / 6 (0.00%) 0	1 / 6 (16.67%) 1	0 / 2 (0.00%) 0
Blood and lymphatic system disorders Leukocytosis subjects affected / exposed occurrences (all)	0 / 6 (0.00%) 0	0 / 6 (0.00%) 0	1 / 2 (50.00%) 1
Ear and labyrinth disorders Ear pain subjects affected / exposed occurrences (all)	0 / 6 (0.00%) 0	0 / 6 (0.00%) 0	1 / 2 (50.00%) 1
Tympanic membrane disorder subjects affected / exposed occurrences (all)	0 / 6 (0.00%) 0	0 / 6 (0.00%) 0	1 / 2 (50.00%) 1
Tympanic membrane hyperaemia subjects affected / exposed occurrences (all)	0 / 6 (0.00%) 0	0 / 6 (0.00%) 0	1 / 2 (50.00%) 1
Eye disorders Blepharospasm subjects affected / exposed occurrences (all)	1 / 6 (16.67%) 1	0 / 6 (0.00%) 0	0 / 2 (0.00%) 0
Gastrointestinal disorders Abdominal pain			

subjects affected / exposed	1 / 6 (16.67%)	0 / 6 (0.00%)	0 / 2 (0.00%)
occurrences (all)	1	0	0
Abdominal pain upper			
subjects affected / exposed	0 / 6 (0.00%)	1 / 6 (16.67%)	0 / 2 (0.00%)
occurrences (all)	0	1	0
Abnormal faeces			
subjects affected / exposed	0 / 6 (0.00%)	0 / 6 (0.00%)	1 / 2 (50.00%)
occurrences (all)	0	0	1
Diarrhoea			
subjects affected / exposed	0 / 6 (0.00%)	0 / 6 (0.00%)	0 / 2 (0.00%)
occurrences (all)	0	0	0
Eructation			
subjects affected / exposed	1 / 6 (16.67%)	0 / 6 (0.00%)	0 / 2 (0.00%)
occurrences (all)	1	0	0
Gingival erythema			
subjects affected / exposed	0 / 6 (0.00%)	0 / 6 (0.00%)	0 / 2 (0.00%)
occurrences (all)	0	0	0
Gingival swelling			
subjects affected / exposed	0 / 6 (0.00%)	0 / 6 (0.00%)	0 / 2 (0.00%)
occurrences (all)	0	0	0
Nausea			
subjects affected / exposed	0 / 6 (0.00%)	0 / 6 (0.00%)	1 / 2 (50.00%)
occurrences (all)	0	0	1
Vomiting			
subjects affected / exposed	0 / 6 (0.00%)	2 / 6 (33.33%)	1 / 2 (50.00%)
occurrences (all)	0	3	1
Skin and subcutaneous tissue disorders			
Erythema			
subjects affected / exposed	1 / 6 (16.67%)	0 / 6 (0.00%)	0 / 2 (0.00%)
occurrences (all)	1	0	0
Hyperhidrosis			
subjects affected / exposed	1 / 6 (16.67%)	0 / 6 (0.00%)	0 / 2 (0.00%)
occurrences (all)	1	0	0
Livedo reticularis			
subjects affected / exposed	0 / 6 (0.00%)	0 / 6 (0.00%)	1 / 2 (50.00%)
occurrences (all)	0	0	1

Pruritus subjects affected / exposed occurrences (all)	1 / 6 (16.67%) 1	0 / 6 (0.00%) 0	0 / 2 (0.00%) 0
Renal and urinary disorders Dysuria subjects affected / exposed occurrences (all)	0 / 6 (0.00%) 0	0 / 6 (0.00%) 0	0 / 2 (0.00%) 0
Musculoskeletal and connective tissue disorders Arthralgia subjects affected / exposed occurrences (all)  Back pain subjects affected / exposed occurrences (all)  Pain in extremity subjects affected / exposed occurrences (all)	0 / 6 (0.00%) 0  0 / 6 (0.00%) 0  0 / 6 (0.00%) 0	0 / 6 (0.00%) 0  1 / 6 (16.67%) 1  0 / 6 (0.00%) 0	0 / 2 (0.00%) 0  0 / 2 (0.00%) 0  0 / 2 (0.00%) 0
Infections and infestations Bullous impetigo subjects affected / exposed occurrences (all)  Ear infection subjects affected / exposed occurrences (all)  Gastroenteritis viral subjects affected / exposed occurrences (all)  Nasopharyngitis subjects affected / exposed occurrences (all)  Otitis media subjects affected / exposed occurrences (all)  Tooth abscess subjects affected / exposed occurrences (all)	0 / 6 (0.00%) 0  0 / 6 (0.00%) 0  1 / 6 (16.67%) 1  1 / 6 (16.67%) 1  0 / 6 (0.00%) 0  0 / 6 (0.00%) 0	0 / 6 (0.00%) 0  0 / 6 (0.00%) 0  0 / 6 (0.00%) 0  0 / 6 (0.00%) 0  0 / 6 (0.00%) 0	0 / 2 (0.00%) 0  0 / 2 (0.00%) 0  0 / 2 (0.00%) 0  1 / 2 (50.00%) 1  0 / 2 (0.00%) 0  0 / 2 (0.00%) 0

Upper respiratory tract infection subjects affected / exposed occurrences (all)	0 / 6 (0.00%) 0	0 / 6 (0.00%) 0	0 / 2 (0.00%) 0
Viral upper respiratory tract infection subjects affected / exposed occurrences (all)	0 / 6 (0.00%) 0	1 / 6 (16.67%) 1	0 / 2 (0.00%) 0
Metabolism and nutrition disorders Decreased appetite subjects affected / exposed occurrences (all)	0 / 6 (0.00%) 0	0 / 6 (0.00%) 0	0 / 2 (0.00%) 0



## More information

### Substantial protocol amendments (globally)

Were there any global substantial amendments to the protocol? Yes

Date	Amendment
31 May 2018	<ul style="list-style-type: none"><li>- Information on the new WVE-210201 liquid formulation was provided</li><li>- GGT was specified in the clinical laboratory panel for evaluation as a potential marker of changes in liver function in patients with DMD</li><li>- Administrative language regarding withdrawal, discontinuations, un-blinding, randomization, and study termination was clarified</li><li>- Language regarding reconsent once patients reach legal age was added</li></ul>

Notes:

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### Interruptions (globally)

Were there any global interruptions to the trial? No

### Limitations and caveats

None reported