Clinical trial results:
A 24 week Randomized Double-Blind, Placebo-Controlled Study followed by 72 week open-label extension to assess the efficacy, safety and tolerability of drisapersen sodium in subjects with Duchenne Muscular Dystrophy

Summary

EudraCT number	2014-005296-81
Trial protocol	BE
Global end of trial date	09 September 2016

Results information

Result version number	v1 (current)
This version publication date	30 December 2018
First version publication date	30 December 2018
Summary attachment (see zip file)	BMN-051-303 statement (BMN-051-303 statement.pdf)

Trial information

Trial identification

Sponsor protocol code	BMN-051-303

Additional study identifiers

ISRCTN number	-
ClinicalTrials.gov id (NCT number)	-
WHO universal trial number (UTN)	-
Other trial identifiers	Duchenne Muscular Dystrophy : DMD

Notes:

Sponsors

Sponsor organisation name	BioMarin Pharmaceutical Inc.
Sponsor organisation address	105 Digital Drive, Novato, United States, CA 94949
Public contact	Clinical Trials Information, BioMarin Pharmaceutical Inc, clinicaltrials@bmrn.com
Scientific contact	Clinical Trials Information, BioMarin Pharmaceutical Inc, clinicaltrials@bmrn.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	09 September 2016
Is this the analysis of the primary completion data?	No

Global end of trial reached?	Yes
Global end of trial date	09 September 2016
Was the trial ended prematurely?	Yes

Notes:

General information about the trial

Main objective of the trial:

• To investigate the efficacy of drisapersen sodium administered for 24 weeks compared to a placebo control group in ambulant subjects with DMD.

Protection of trial subjects:

Not applicable

Background therapy: -

Evidence for comparator: -

Actual start date of recruitment	01 December 2015
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
Worldwide total number of subjects	1
EEA total number of subjects	1

Notes:

Subjects enrolled per age group

In utero	0
Preterm newborn - gestational age < 37 wk	0
Newborns (0-27 days)	0
Infants and toddlers (28 days-23 months)	0
Children (2-11 years)	1
Adolescents (12-17 years)	0
Adults (18-64 years)	0
From 65 to 84 years	0
85 years and over	0

Subject disposition

Recruitment

Recruitment details: -

Pre-assignment

Screening details:

Not applicable - no subjects enrolled

Period 1

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

Blinding implementation details:

NA

Arms

Arm title	Not applicable

Arm description:

NA - no subjects enrolled

Arm type	NA
Investigational medicinal product name	Not applicable
Investigational medicinal product code	
Other name	
Pharmaceutical forms	Solution for infusion
Routes of administration	Intravenous use

Dosage and administration details:

No subjects enrolled - no doses given in study

Number of subjects in period 1	Not applicable
Started	1
Completed	1

Baseline characteristics

End points

End points reporting groups

Reporting group title	Not applicable
Reporting group description: NA - no subjects enrolled	
Subject analysis set title	Not applicable
Subject analysis set type	Per protocol
Subject analysis set description: Not applicable - no subjects enrolled	

Primary: Not applicable

End point title	Not applicable[1]
End point description: No applicable	
End point type	Primary
End point timeframe: Not applicable	

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: Not applicable - no subjects were enrolled

End point values	Not applicable			
Subject group type	Reporting group			
Number of subjects analysed	1[2]			
Units: subject	1			

Notes:

[2] - Not applicable

Statistical analyses

No statistical analyses for this end point

Adverse events

Adverse events information[1]	
Timeframe for reporting adverse events:	
Not applicable - no subjects anrolled	
Assessment type	Systematic

Dictionary used	
Dictionary name	MedDRA
Dictionary version	9

Frequency threshold for reporting non-serious adverse events: 1 %

Notes:

[1] - There are no non-serious adverse events recorded for these results. It is expected that there will be at least one non-serious adverse event reported.
Justification: Not applicable - no subjects were enrolled

More information

Substantial protocol amendments (globally)

Were there any global substantial amendments to the protocol? No

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.

The trial was ended before any subjects were enrolled.

No results are available for posting.

Notes: