



Clinical trial results:

Observational study of clinical outcomes for testosterone treatment of pubertal delay in Duchenne Muscular Dystrophy.

Summary

EudraCT number	2015-003195-68
Trial protocol	GB
Global end of trial date	04 March 2019

Results information

Result version number	v1 (current)
This version publication date	05 December 2021
First version publication date	05 December 2021
Summary attachment (see zip file)	EJE Testosterone study paper (TestosteronestudyEJE200709-2.pdf)

Trial information

Trial identification

Sponsor protocol code	2015-003195-68
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Additional study identifiers

ISRCTN number	-
ClinicalTrials.gov id (NCT number)	NCT02571205
WHO universal trial number (UTN)	-

Notes:

Sponsors

Sponsor organisation name	Newcastle Upon Tyne Hospitals Foundation Trust
Sponsor organisation address	Queen Victoria Road, Newcastle Upon Tyne, United Kingdom,
Public contact	Prof Volker Straub, Newcastle University, +44 01912418663, volker.straub@ncl.ac.uk
Scientific contact	Prof Volker Straub, Newcastle University, +44 01912418663, volker.straub@ncl.ac.uk

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	30 September 2020
Is this the analysis of the primary completion data?	Yes
Primary completion date	04 March 2019
Global end of trial reached?	Yes
Global end of trial date	04 March 2019
Was the trial ended prematurely?	No

Notes:

General information about the trial

Main objective of the trial:

Evaluate patient satisfaction in response to testosterone replacement therapy in DMD patients with pubertal delay.

Protection of trial subjects:

All data was reviewed regularly by the data monitoring committee as well as the trial steering group. The CTU also performed regular audits.

Background therapy: -

Evidence for comparator: -

Actual start date of recruitment	01 October 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	United Kingdom: 15
Worldwide total number of subjects	15
EEA total number of subjects	15

Notes:

Subjects enrolled per age group

In utero	0
Preterm newborn - gestational age < 37 wk	0
Newborns (0-27 days)	0
Infants and toddlers (28 days-23 months)	0
Children (2-11 years)	0
Adolescents (12-17 years)	15
Adults (18-64 years)	0
From 65 to 84 years	0
85 years and over	0

Subject disposition

Recruitment

Recruitment details:

15 patients recruited, aged between 12 and 16.9 years of age

Pre-assignment

Screening details:

16 patients screened.

Period 1

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

Arms

Arm title	Single arm study
Arm description: -	
Arm type	Experimental
Investigational medicinal product name	Sustanon
Investigational medicinal product code	
Other name	
Pharmaceutical forms	Emulsion for injection
Routes of administration	Injection

Dosage and administration details:

incremental regimen, 4-weekly injection for 2 years

Number of subjects in period 1	Single arm study
Started	15
Completed	15

Baseline characteristics

End points

End points reporting groups

Reporting group title	Single arm study
Reporting group description: -	

Primary: Score on TSQM at end of study

End point title	Score on TSQM at end of study ^[1]
End point description:	

End point type	Primary
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End point timeframe:

2 years

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 performed- descriptive only

End point values	Single arm study			
Subject group type	Reporting group			
Number of subjects analysed	15			
Units: integer	15			

Statistical analyses

No statistical analyses for this end point

Adverse events

Adverse events information

Timeframe for reporting adverse events:

study period

Assessment type	Systematic
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Dictionary used

Dictionary name	MedDRA
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Dictionary version	118.1
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Reporting groups

Reporting group title	Main study group
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Reporting group description: -

Serious adverse events	Main study group		
Total subjects affected by serious adverse events			
subjects affected / exposed	5 / 15 (33.33%)		
number of deaths (all causes)	0		
number of deaths resulting from adverse events			
Gastrointestinal disorders			
Vomiting			
subjects affected / exposed	1 / 15 (6.67%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Diarrhoea infectious			
subjects affected / exposed	1 / 15 (6.67%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Musculoskeletal and connective tissue disorders			
lower limb fracture			
subjects affected / exposed	2 / 15 (13.33%)		
occurrences causally related to treatment / all	0 / 3		
deaths causally related to treatment / all	0 / 0		
planned Achilles tendon release			
subjects affected / exposed	1 / 15 (6.67%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		

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

Non-serious adverse events	Main study group		
Total subjects affected by non-serious adverse events			
subjects affected / exposed	15 / 15 (100.00%)		
Injury, poisoning and procedural complications			
Choking sensation			
subjects affected / exposed	1 / 15 (6.67%)		
occurrences (all)	1		
Nervous system disorders			
Headache			
subjects affected / exposed	2 / 15 (13.33%)		
occurrences (all)	2		
Blood and lymphatic system disorders			
Epistaxis			
subjects affected / exposed	1 / 15 (6.67%)		
occurrences (all)	1		
Immune system disorders			
Rhinitis allergic			
subjects affected / exposed	1 / 15 (6.67%)		
occurrences (all)	1		
Gastrointestinal disorders			
Diarrhoea			
subjects affected / exposed	1 / 15 (6.67%)		
occurrences (all)	1		
Dehydration			
subjects affected / exposed	1 / 15 (6.67%)		
occurrences (all)	1		
Constipation			
subjects affected / exposed	1 / 15 (6.67%)		
occurrences (all)	1		
gallstones			

subjects affected / exposed	1 / 15 (6.67%)		
occurrences (all)	1		
Abdominal pain			
subjects affected / exposed	1 / 15 (6.67%)		
occurrences (all)	1		
Respiratory, thoracic and mediastinal disorders			
Cough			
subjects affected / exposed	1 / 15 (6.67%)		
occurrences (all)	1		
Upper respiratory tract infection			
subjects affected / exposed	4 / 15 (26.67%)		
occurrences (all)	7		
Lower respiratory tract infection			
subjects affected / exposed	3 / 15 (20.00%)		
occurrences (all)	4		
Skin and subcutaneous tissue disorders			
Injection related reaction			
subjects affected / exposed	1 / 15 (6.67%)		
occurrences (all)	1		
Acne			
subjects affected / exposed	1 / 15 (6.67%)		
occurrences (all)	1		
Chillblains			
subjects affected / exposed	1 / 15 (6.67%)		
occurrences (all)	1		
Pilonidal cyst			
subjects affected / exposed	1 / 15 (6.67%)		
occurrences (all)	1		
Psoriasis			
subjects affected / exposed	1 / 15 (6.67%)		
occurrences (all)	1		
Rash			
subjects affected / exposed	1 / 15 (6.67%)		
occurrences (all)	1		
Haemorrhoids			

<p>subjects affected / exposed</p> <p>occurrences (all)</p> <p>Dry skin</p> <p>subjects affected / exposed</p> <p>occurrences (all)</p> <p>Fungal infection</p> <p>subjects affected / exposed</p> <p>occurrences (all)</p> <p>portacath fitting</p> <p>subjects affected / exposed</p> <p>occurrences (all)</p>	<p>1 / 15 (6.67%)</p> <p>1</p> <p>2 / 15 (13.33%)</p> <p>2</p> <p>2 / 15 (13.33%)</p> <p>2</p> <p>1 / 15 (6.67%)</p> <p>1</p>		
<p>Renal and urinary disorders</p> <p>Bladder dysfunction</p> <p>subjects affected / exposed</p> <p>occurrences (all)</p> <p>Acute kidney injury</p> <p>subjects affected / exposed</p> <p>occurrences (all)</p> <p>Urinary tract infection</p> <p>subjects affected / exposed</p> <p>occurrences (all)</p>	<p>1 / 15 (6.67%)</p> <p>1</p> <p>1 / 15 (6.67%)</p> <p>1</p> <p>1 / 15 (6.67%)</p> <p>1</p>		
<p>Musculoskeletal and connective tissue disorders</p> <p>vertebral fracture</p> <p>subjects affected / exposed</p> <p>occurrences (all)</p> <p>Lower limb fracture</p> <p>subjects affected / exposed</p> <p>occurrences (all)</p> <p>reposition femoral nail</p> <p>subjects affected / exposed</p> <p>occurrences (all)</p> <p>Back pain</p> <p>subjects affected / exposed</p> <p>occurrences (all)</p> <p>Muscle spasms</p>	<p>5 / 15 (33.33%)</p> <p>6</p> <p>3 / 15 (20.00%)</p> <p>6</p> <p>1 / 15 (6.67%)</p> <p>1</p> <p>4 / 15 (26.67%)</p> <p>4</p>		

subjects affected / exposed	1 / 15 (6.67%)		
occurrences (all)	1		
Upper limb fracture			
subjects affected / exposed	2 / 15 (13.33%)		
occurrences (all)	2		
Fall			
subjects affected / exposed	2 / 15 (13.33%)		
occurrences (all)	6		
ankle injury			
subjects affected / exposed	1 / 15 (6.67%)		
occurrences (all)	1		
foot injury			
subjects affected / exposed	3 / 15 (20.00%)		
occurrences (all)	3		
bilateral achilles tendon release			
subjects affected / exposed	2 / 15 (13.33%)		
occurrences (all)	2		
Infections and infestations			
Pyrexia			
subjects affected / exposed	1 / 15 (6.67%)		
occurrences (all)	2		
Tonsillitis streptococcal			
subjects affected / exposed	1 / 15 (6.67%)		
occurrences (all)	1		
Otitis media			
subjects affected / exposed	1 / 15 (6.67%)		
occurrences (all)	1		
Vomiting			
subjects affected / exposed	1 / 15 (6.67%)		
occurrences (all)	1		

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

None reported