Clinical trial results:
Can local intramuscular botulinum toxin improve dysphagia in patients with myopathic dysphagia and constriction of the cricoid muscle?

Summary

EudraCT number	2014-002210-23
Trial protocol	DK
Global end of trial date	28 November 2018

Results information

Result version number	v1 (current)
This version publication date	08 January 2021
First version publication date	08 January 2021

Trial information

Trial identification

Sponsor protocol code	1.4botox

Additional study identifiers

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

Notes:

Sponsors

Sponsor organisation name	Rigshospitalet
Sponsor organisation address	BLEGDAMSVEJ 9, København Ø, Denmark,
Public contact	Neurology 2081, Rigshospitalet, 45 35459674, nanna.witting@regionh.dk
Scientific contact	Neurology 2081, Rigshospitalet, 45 35459674, nanna.witting@regionh.dk

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	19 November 2020
Is this the analysis of the primary completion data?	Yes
Primary completion date	28 November 2018
Global end of trial reached?	Yes
Global end of trial date	28 November 2018
Was the trial ended prematurely?	No

Notes:

General information about the trial

Main objective of the trial:

To investigate if local intramuscular botulinum toxin can alleviate myopahtic dysphagia

Protection of trial subjects:

None

Background therapy:

One had prednisolone

Evidence for comparator: -

Actual start date of recruitment	01 July 2014
Long term follow-up planned	No
Independent data monitoring committee (IDMC) involvement?	No

Notes:

Population of trial subjects

Subjects enrolled per country

Country: Number of subjects enrolled	Denmark: 13
Worldwide total number of subjects	13
EEA total number of subjects	13

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)	0
Adults (18-64 years)	0
From 65 to 84 years	13
85 years and over	0

Subject disposition

Recruitment

Recruitment details:

From own clinic and collaborators.

Pre-assignment

Screening details:

Dysphagia score

Period 1

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

Arms

Arm title	Dysphagiascore

Arm description:

Three ascending doses og drug.

Arm type	Active comparator
Investigational medicinal product name	botulinum toxin A
Investigational medicinal product code	
Other name	
Pharmaceutical forms	Injection
Routes of administration	Intramuscular use

Dosage and administration details:

5-25 units in the cricopharyngeal muscle bilaterally

Number of subjects in period 1	Dysphagiascore
Started	13
12w1 dysphagia score	13
Completed	13

Baseline characteristics

Reporting groups

Reporting group title	Treatment period

Reporting group description: -

Reporting group values	Treatment period	Total	
Number of subjects	13	13	
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)	0	0	
Adolescents (12-17 years)	0	0	
Adults (18-64 years)	0	0	
From 65-84 years	13	13	
85 years and over	0	0	
Gender categorical Units: Subjects			
Female	4	4	
Male	9	9	

Subject analysis sets

Subject analysis set title	Best 12W dyspahgia score or latest measurement
Subject analysis set type	Intention-to-treat

Subject analysis set description:

Best 12W dyspahgia score or latest measurement compared to baseline

Reporting group values	Best 12W dyspahgia score or latest measurement		
Number of subjects	13		
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)	0		
Adolescents (12-17 years)	0		
Adults (18-64 years)	0		
From 65-84 years	13		
85 years and over	0		

Gender categorical Units: Subjects			
Female	4		
Male	9		

End points

End points reporting groups

Reporting group title	Dysphagiascore
Reporting group description: Three ascending doses og drug.	
Subject analysis set title	Best 12W dyspahgia score or latest measurement
Subject analysis set type	Intention-to-treat
Subject analysis set description: Best 12W dyspahgia score or latest measurement compared to baseline	

Primary: Best 12w dysphagia score or latest measurement compared to baseline

End point title	Best 12w dysphagia score or latest measurement compared to baseline[1]
End point description:	
End point type	Primary
End point timeframe: Best 12w dysphagia score or latest measurement	

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: An Attachment with raw data and statistical analysis is provided.

End point values	Dysphagiascore	Best 12W dyspahgia score or latest measurement		
Subject group type	Reporting group	Subject analysis set		
Number of subjects analysed	13	13		
Units: dysphagia score	13	13		

Attachments (see zip file)	Botox results/Resultat EUDRACT 20122020.xlsx

Statistical analyses

No statistical analyses for this end point

Adverse events

Adverse events information[1]	
Timeframe for reporting adverse events: 12 weeks after last injection.	
Assessment type	Systematic

Dictionary used	
Dictionary name	none
Dictionary version	1

Frequency threshold for reporting non-serious adverse events: 0 %

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: No non-serious adverse events were reported. One person had a rash after injection.

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