



Clinical trial results:

Phase IIa study to characterize the effects of the Spiegelmer® NOX-H94 on anemia of chronic disease in patients with cancer

Summary

EudraCT number	2012-001525-27
Trial protocol	AT BG
Global end of trial date	30 December 2013

Results information

Result version number	v1 (current)
This version publication date	03 February 2016
First version publication date	30 July 2015

Trial information

Trial identification

Sponsor protocol code	SNOXH94C201
-----------------------	-------------

Additional study identifiers

ISRCTN number	-
ClinicalTrials.gov id (NCT number)	NCT01691040
WHO universal trial number (UTN)	-

Notes:

Sponsors

Sponsor organisation name	NOXXON Pharma AG
Sponsor organisation address	Max-Dohrn Strasse 8-10, Berlin, Germany, 10589
Public contact	Clinical Trial Disclosure Desk NOXXON, NOXXON Pharma AG, clinicaltrialdisclosuredesk@noxxon.com
Scientific contact	Clinical Trial Disclosure Desk NOXXON, NOXXON Pharma AG, clinicaltrialdisclosuredesk@noxxon.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	29 April 2014
Is this the analysis of the primary completion data?	Yes
Primary completion date	30 December 2013
Global end of trial reached?	Yes
Global end of trial date	30 December 2013
Was the trial ended prematurely?	No

Notes:

General information about the trial

Main objective of the trial:

To assess the response rate of anemia in patients with cancer to treatment with NOX-H94.

Protection of trial subjects:

This clinical study was written, conducted and reported in accordance with the protocol, ICH GCP Guidelines, and applicable local regulations, including the European Directive 2001/20/EC, 2005/28/EC, and 2003/63/EC and relevant national and local legislations, and with the ethical principles that have their origin in the Declaration of Helsinki. Only subjects that met all the study inclusion and none of the exclusion criteria were randomized. Study drug administrations were performed by qualified and trained study personnel. Patient who received treatment were closely followed by means of adverse event reporting and vital signs. In the event of a study related adverse event, patient would have been monitored to determine the outcome. The clinical course of the AE were followed up according to accepted standards of medical practice, even after the end of the period of observation, until a satisfactory explanation is found or the Investigator considers it medically justifiable to terminate follow-up.

Background therapy: -

Evidence for comparator: -

Actual start date of recruitment	25 September 2012
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	Romania: 7
Country: Number of subjects enrolled	Bulgaria: 5
Worldwide total number of subjects	12
EEA total number of subjects	12

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)	6
From 65 to 84 years	6
85 years and over	0

Subject disposition

Recruitment

Recruitment details: -

Pre-assignment

Screening details:

A total of 33 patients were screened and thereof 21 were screen failure.

After a screening period of maximum 28 days patients started to be treated.

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	Lexaptepid
Arm description: -	
Arm type	Experimental
Investigational medicinal product name	Lexaptepid Pegol
Investigational medicinal product code	NOX-H94
Other name	
Pharmaceutical forms	Solution for injection
Routes of administration	Intravenous use

Dosage and administration details:

Patients were treated twice weekly with intravenous (i.v.) doses of 1.2 mg/kg Lexaptepid Pegol administered by slow injection over 1 minute. Nine doses were administered over a treatment period of 4 weeks.

Formulation was a preservative-free, sterile solution in an aqueous citrate buffer containing sucrose.

Number of subjects in period 1	Lexaptepid
Started	12
Completed	11
Not completed	1
Consent withdrawn by subject	1

Baseline characteristics

Reporting groups

Reporting group title

Overall trial

Reporting group description: -

Reporting group values	Overall trial	Total	
Number of subjects	12	12	
Age categorical			
Units: Subjects			
Adults (18-64 years)	6	6	
From 65-84 years	6	6	
Age continuous			
Units: years			
arithmetic mean	62		
standard deviation	± 14	-	
Gender categorical			
Units: Subjects			
Female	7	7	
Male	5	5	

End points

End points reporting groups

Reporting group title	Lexaptepid
Reporting group description: -	

Primary: Response Rate

End point title	Response Rate ^[1]
-----------------	------------------------------

End point description:

Treatment responders were defined by Hb increase ≥ 1 g/dL OR reticulocyte index (RI) normalization ($\geq 1\%$) at any time point until 1 week after the end of treatment AND absence of all of the following treatment failure criteria until 1 week after the end of treatment:

- Erythrocyte transfusion, ESA or i.v. iron
- Hb drop by ≥ 1 g/dL
- Treatment interruption due to adverse events (AEs)

End point type	Primary
----------------	---------

End point timeframe:

Any time point until 1 week after the end of treatment.

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: The primary efficacy endpoint was assessed descriptively only, no statistical analysis has been performed.

For the primary endpoint only "responder: yes / no" was assessed according to the end point description above and counted.

End point values	Lexaptepid			
Subject group type	Reporting group			
Number of subjects analysed	12			
Units: responders				
Treatment Responders	5			

Statistical analyses

No statistical analyses for this end point

Adverse events

Adverse events information

Timeframe for reporting adverse events:

Baseline (Day 1) until end of follow up (Day 85)

Assessment type	Systematic
-----------------	------------

Dictionary used

Dictionary name	MedDRA
-----------------	--------

Dictionary version	15.1
--------------------	------

Reporting groups

Reporting group title	Lexaptepid
-----------------------	------------

Reporting group description: -

Serious adverse events	Lexaptepid		
Total subjects affected by serious adverse events			
subjects affected / exposed	1 / 12 (8.33%)		
number of deaths (all causes)	0		
number of deaths resulting from adverse events	0		
Metabolism and nutrition disorders			
Dehydration			
subjects affected / exposed	1 / 12 (8.33%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		

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

Non-serious adverse events	Lexaptepid		
Total subjects affected by non-serious adverse events			
subjects affected / exposed	8 / 12 (66.67%)		
Investigations			
Transaminases increased			
subjects affected / exposed	1 / 12 (8.33%)		
occurrences (all)	1		
Injury, poisoning and procedural complications			
Chemical burn of skin			
subjects affected / exposed	1 / 12 (8.33%)		
occurrences (all)	1		
Vascular disorders			

Hypotension subjects affected / exposed occurrences (all)	1 / 12 (8.33%) 1		
Orthostatic hypotension subjects affected / exposed occurrences (all)	2 / 12 (16.67%) 3		
Nervous system disorders Dizziness subjects affected / exposed occurrences (all)	2 / 12 (16.67%) 2		
Headache subjects affected / exposed occurrences (all)	1 / 12 (8.33%) 1		
Blood and lymphatic system disorders Neutropenia subjects affected / exposed occurrences (all)	1 / 12 (8.33%) 1		
Respiratory, thoracic and mediastinal disorders Cough subjects affected / exposed occurrences (all)	1 / 12 (8.33%) 1		
Psychiatric disorders Agitation subjects affected / exposed occurrences (all)	1 / 12 (8.33%) 1		
Insomnia subjects affected / exposed occurrences (all)	1 / 12 (8.33%) 1		
Musculoskeletal and connective tissue disorders Back pain subjects affected / exposed occurrences (all)	1 / 12 (8.33%) 1		
Infections and infestations Pyelonephritis acute ¹ subjects affected / exposed occurrences (all)	1 / 12 (8.33%) 1		
Soft tissue infection			

subjects affected / exposed	1 / 12 (8.33%)		
occurrences (all)	1		
Viral infection			
subjects affected / exposed	1 / 12 (8.33%)		
occurrences (all)	1		

More information

Substantial protocol amendments (globally)

Were there any global substantial amendments to the protocol? Yes

Date	Amendment
18 February 2013	<p>AT/BG: Amendment 1:</p> <p>The study population and few inclusion/exclusion criteria were modified:</p> <ul style="list-style-type: none">- The study population was enlarged to anemic patients with solid tumors. This change required also an update of the study title.- The cut-off value for TSAT was increased to 50% in accordance to the new definition of functional iron deficiency from the NCCN Guideline 1.2013.- The criterion on previous treatment with systemic anti-cancer therapy was deleted. <p>The following exclusion criteria were added and protocol sections modified:</p> <ul style="list-style-type: none">- A known or suspected chronic bleeding.- Patients with tumor with gastro-intestinal involvement, without a negative test for fecal occult blood. The test was added to the screening procedures in this kind of patients.- Selection and withdrawal of patients (Section 7.1): The possibility to re-screen patients after an appropriate interval was introduced, in case the patient did not meet the selection criteria at the first screening visit.
27 May 2013	<p>RO: Amendment 1:</p> <p>The study population and few inclusion/exclusion criteria were modified:</p> <ul style="list-style-type: none">- The study population was enlarged to anemic patients with solid tumors. This change required also an update of the study title.- The cut-off value for TSAT was increased to 20% in accordance to the new definition of functional iron deficiency from the NCCN Guideline 1.2013.- The criterion on previous treatment with systemic anti-cancer therapy was deleted. <p>The following exclusion criteria were added and protocol sections modified:</p> <ul style="list-style-type: none">- A known or suspected chronic bleeding.- Patients with tumor with gastro-intestinal involvement, without a negative test for fecal occult blood. The test was added to the screening procedures in this kind of patients.- Selection and withdrawal of patients (Section 7.1): The possibility to re-screen patients after an appropriate interval was introduced, in case the patient did not meet the selection criteria at the first screening visit. <p>Exclusion of patients with tumor of the gastrointestinal tract and of the liver (metastatic or primary); for this reason the fecal blood test was not required anymore.</p>

Notes:

Interruptions (globally)

Were there any global interruptions to the trial? No

Limitations and caveats

None reported