



## Clinical trial results:

**Single-center, double-blind, randomized, placebo-controlled, 2-period/2-treatment crossover study investigating the effect of miglustat on the nasal potential difference in patients with cystic fibrosis homozygous for the F508 mutation**

### Summary

EudraCT number	2006-002049-35
Trial protocol	ES
Global end of trial date	22 February 2008

### Results information

Result version number	v2 (current)
This version publication date	07 November 2019
First version publication date	06 August 2015
Version creation reason	<ul style="list-style-type: none"><li>• Correction of full data set</li><li>Change of Sponsor</li></ul>

### Trial information

#### Trial identification

Sponsor protocol code	AC-056-201
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#### Additional study identifiers

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

Notes:

### Sponsors

Sponsor organisation name	Idorsia Pharmaceuticals Ltd
Sponsor organisation address	Hegenheimermattweg 91, Allschwil, Switzerland, 4123
Public contact	Clinical Trials Disclosure Desk, Idorsia Pharmaceuticals Ltd, clinical-trials-disclosure@idorsia.com
Scientific contact	Clinical Trials Disclosure Desk, Idorsia Pharmaceuticals Ltd, clinical-trials-disclosure@idorsia.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	12 February 2009
Is this the analysis of the primary completion data?	Yes
Primary completion date	22 February 2008
Global end of trial reached?	Yes
Global end of trial date	22 February 2008
Was the trial ended prematurely?	Yes

Notes:

## General information about the trial

Main objective of the trial:

To demonstrate that miglustat restores the function of the cystic fibrosis transmembrane conductance regulator (CFTR) in patients with cystic fibrosis homozygous for the  $\Delta F508$  mutation, as reflected in nasal potential difference (NPD)

Protection of trial subjects:

Prior to the start of the trial the study center consulted an Independent Ethics Committee (IEC), i.e., a review panel that was responsible for ensuring the protection of the rights, safety, and well-being of human patients involved in a clinical investigation. The sponsor ensured that the IEC consulted was adequately constituted to provide assurance of that protection, and maintained a list of committee members and their qualifications. The protocol and any material provided to the patient (such as a subject information sheet or description of the study used to obtain informed consent) were reviewed and approved by the appropriate IEC before the study was started.

This study was conducted in full conformance with the principles of the 'Declaration of Helsinki' and with the laws and regulations of the country in which the research was conducted.

Both Actelion and the investigator had the right to terminate the study at any time, and in such a case, were responsible for protecting the patients' interests.

Written informed consent was obtained from each individual participating in the study prior to any study procedure and after adequate explanation of the aims, methods, objectives, and potential hazards of the study. It was made clear to each patient that he or she was completely free to refuse to enter the study, or to withdraw from it at any time for any reason. A description of any incentives to participate in the study was provided in the informed consent form.

Background therapy:

Miglustat or placebo was given on top of standard care. Restrictions were applied for other investigational drugs and/or therapies, e.g., gene therapy.

Evidence for comparator: -

Actual start date of recruitment	06 November 2007
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	Spain: 3
Worldwide total number of subjects	3
EEA total number of subjects	3

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)	0
Adolescents (12-17 years)	3
Adults (18-64 years)	0
From 65 to 84 years	0
85 years and over	0

## Subject disposition

### Recruitment

Recruitment details:

One investigational center in Spain. The study was terminated prematurely after only 3 patients had participated in the study. One reason for premature study termination was low recruitment.

### Pre-assignment

Screening details:

Screening examinations were done within 3 weeks and 3 days prior to the first administration of study medication.

### Period 1

Period 1 title	Baseline Period
Is this the baseline period?	Yes
Allocation method	Randomised - controlled
Blinding used	Double blind
Roles blinded	Subject, Investigator, Monitor, Data analyst

Blinding implementation details:

The investigator and study staff, the patients, monitors, and the sponsor staff remained blinded to the treatment until study closure. The investigational drug and its matching placebo were indistinguishable and all patient kits were packaged in the same way.

### Arms

Arm title	Miglustat 200 mg t.i.d.
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Arm description:

Miglustat 200 mg t.i.d.

Arm type	Experimental
Investigational medicinal product name	Miglustat 200 mg t.i.d.
Investigational medicinal product code	
Other name	
Pharmaceutical forms	Capsule
Routes of administration	Oral use

Dosage and administration details:

Miglustat 200 mg t.i.d. capsules, oral use

<b>Number of subjects in period 1</b>	Miglustat 200 mg t.i.d.
Started	3
Completed	3

## Period 2

Period 2 title	Period 1
Is this the baseline period?	No
Allocation method	Randomised - controlled
Blinding used	Double blind
Roles blinded	Subject, Investigator, Monitor, Data analyst

Blinding implementation details:

The investigator and study staff, the patients, monitors, and the sponsor staff remained blinded to the treatment until study closure. The investigational drug and its matching placebo were indistinguishable and all patient kits were packaged in the same way.

## Arms

Arm title	Miglustat 200 mg t.i.d.
Arm description: Miglustat 200 mg t.i.d. for 7 days	
Arm type	Experimental
Investigational medicinal product name	Miglustat 200 mg t.i.d.
Investigational medicinal product code	
Other name	
Pharmaceutical forms	Capsule
Routes of administration	Oral use

Dosage and administration details:

Miglustat 200 mg t.i.d. capsules, oral use

<b>Number of subjects in period 2</b>	Miglustat 200 mg t.i.d.
Started	3
Completed	3

## Period 3

Period 3 title	Period 2
Is this the baseline period?	No
Allocation method	Randomised - controlled
Blinding used	Double blind
Roles blinded	Subject, Investigator, Monitor, Data analyst

Blinding implementation details:

The investigator and study staff, the patients, monitors, and the sponsor staff remained blinded to the treatment until study closure. The investigational drug and its matching placebo were indistinguishable and all patient kits were packaged in the same way.

## Arms

<b>Arm title</b>	Placebo
Arm description: Placebo 200 mg t.i.d.	
Arm type	Placebo
Investigational medicinal product name	Placebo 200 mg t.i.d.
Investigational medicinal product code	
Other name	
Pharmaceutical forms	Capsule
Routes of administration	Oral use
Dosage and administration details: Matching placebo 200 mg t.i.d. capsules, oral use	

Number of subjects in period 3	Placebo
Started	3
Completed	2
Not completed	1
Consent withdrawn by subject	1

## Baseline characteristics

### Reporting groups

Reporting group title	Baseline Period
Reporting group description:	
Not applicable	

Reporting group values	Baseline Period	Total	
Number of subjects	3	3	
Age categorical			
Units: Subjects			
Age continuous			
Units: years			
arithmetic mean	14.3		
standard deviation	± 4	-	
Gender categorical			
Units:			
Female	1	1	
Male	2	2	

### Subject analysis sets

Subject analysis set title	All-treated set
Subject analysis set type	Full analysis
Subject analysis set description:	
This analysis set includes all randomized patients who received study drug.	

Reporting group values	All-treated set		
Number of subjects	3		
Age categorical			
Units: Subjects			
Age continuous			
Units: years			
arithmetic mean	14.3		
standard deviation	± 4		
Gender categorical			
Units:			
Female	1		
Male	2		

## End points

### End points reporting groups

Reporting group title	Miglustat 200 mg t.i.d.
Reporting group description:	
Miglustat 200 mg t.i.d.	
Reporting group title	Miglustat 200 mg t.i.d.
Reporting group description:	
Miglustat 200 mg t.i.d. for 7 days	
Reporting group title	Placebo
Reporting group description:	
Placebo 200 mg t.i.d.	
Subject analysis set title	All-treated set
Subject analysis set type	Full analysis
Subject analysis set description:	
This analysis set includes all randomized patients who received study drug.	

### Primary: Not applicable

End point title	Not applicable <sup>[1]</sup>
End point description:	
Not 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.

End point values	Miglustat 200 mg t.i.d.	All-treated set		
Subject group type	Reporting group	Subject analysis set		
Number of subjects analysed	0 <sup>[2]</sup>	0 <sup>[3]</sup>		
Units: Not applicable				

Notes:

[2] - Not applicable

[3] - Not applicable

### Statistical analyses

No statistical analyses for this end point

## Adverse events

### Adverse events information

Timeframe for reporting adverse events:

Adverse events from start of Period 1 (Day 1) to end of study (Day 28, Period 2). Serious adverse events from Day -21 until Day 1 (if related to study-mandated procedures) and from Day 1 to Day 56 (Follow up).

Assessment type	Systematic
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### Dictionary used

Dictionary name	MedDRA
Dictionary version	9.0

### Reporting groups

Reporting group title	Overall Period
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Reporting group description:

Period 1 and 2

Serious adverse events	Overall Period		
Total subjects affected by serious adverse events			
subjects affected / exposed	0 / 3 (0.00%)		
number of deaths (all causes)	0		
number of deaths resulting from adverse events	0		

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

Non-serious adverse events	Overall Period		
Total subjects affected by non-serious adverse events			
subjects affected / exposed	1 / 3 (33.33%)		
Gastrointestinal disorders			
Diarrhea			
subjects affected / exposed	1 / 3 (33.33%)		
occurrences (all)	2		

## **More information**

### **Substantial protocol amendments (globally)**

Were there any global substantial amendments to the protocol? No

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### **Interruptions (globally)**

Were there any global interruptions to the trial? No

### **Limitations and caveats**

None reported