- Trial information
- Subject disposition
- Subject analysis sets
- Baseline characteristics
- End points
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- More information

TRIAL INFORMATION

Trial identification

Eudract number	2008-002352-20
Sponsor protocol code	AC-056A202
Full title of trial	Single-center, double-blind, randomized, placebo-controlled, two-period/two-treatment crossover study investigating the effect of miglustat on the nasal potential difference in patients with cystic fibrosis homozygous for the F508del mutation

Additional study identifiers

ISRCTN number	
NCT number	
UTN number	

Sponsor details

Sponsor 1

Name of organisation	Actelion Pharmaceuticals Ltd.
Street address	Gewerbestrasse 16
Town/city	Allschwil
Postal code	4123
Country	Switzerland

SCIENTIFIC CONTACT POINT

Name of organisation	Actelion Pharmaceuticals Ltd.
Functional contact point name	Clinical Trials Disclosure Desk
Telephone number	+()
Email address	clinical-trials-disclosure@actelion.com

PUBLIC CONTACT POINT

Name of organisation	Actelion Pharmaceuticals Ltd.
Functional contact point name	Clinical Trials Disclosure Desk
Telephone number	+()
Email address	clinical-trials-disclosure@actelion.com

Paediatric regulatory details

Is trial part of an agreed paediatric investigation plan (PIP)	no

EMA paediatric investigation plan(s)	
Does article 45 of regulation (EC) no 1901/2006 apply yo this trial?	no
Does article 46 of regulation (EC) no 1901/2006 apply yo this trial?	no

Results analysis stage

Analysis stage	Final
Date of interim/final analysis	2009-03-06
Is this the analysis of the primary completion data?	yes
Primary completion date	2008-12-22
Global end of trial date reached?	yes
Global end of trial date	2008-12-22
Was the trial ended prematurely?	no

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), as reflected in nasal potential difference (NPD), in patients with cystic fibrosis homozygous for the F508del mutation
Actual start date of recruitment	2008-08-26
Long term follow-up planned	no
Independent data monitoring committee (IMDC) involvement?	no
Protection of trial subjects	This study was conducted in full conformance with the principles of the 'Declaration of Helsinki', with the ICH Guidelines on Good Clinical Practice (GCP), and with the laws and regulations of the country in which the research was conducted. 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 subject that he or she was completely free to refuse to enter the study, or to withdraw from it at any time for any reason.
Background therapy	Miglustat or placebo was given on top of standard care. There were no restrictions regarding concomitant medications except that other investigational drugs and/or therapies (e.g., gene therapy) were not allowed.
Evidence for comparator(s)	

Population of trial subjects

TRIAL COUNTRY	PLANNED NUMBER OF SUBJECTS	ACTUAL NUMBER OF SUBJECTS ENROLLED
Belgium		11
Total: worldwide		11
Total: EEA		11

AGE RANGE	PLANNED NUMBER OF SUBJECTS	ACTUAL NUMBER OF SUBJECTS ENROLLED
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)		11

From 65-84 years	0
85 years and over	0

SUBJECT DISPOSITION

Details

Recruitment details	One investigational center in Belgium.
Screening details	A screening examination took place 3–21 days prior to the first administration of study medication.

Pre-assignment period

MILESTONES

Started

Completed

INTERMEDIATE MILESTONES

REASONS FOR NON-COMPLETION

Period 1

Period details

Period title	Baseline period
Is this the baseline period	yes
Allocation method	Randomised - controlled
Blinding type	Double Blind
Roles blinded	Subject, Investigator, Monitor, Data analyst
Blinding details	The investigator and study staff, the patients, the 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.
Are arms mutually exclusive	no
Subjects in period	11

Period arms

TITLE	TYPE	DESCRIPTION	PRODUCTS
Miglustat	Experimental	Miglustat	**Name**: Miglustat 200 mg t.i.d. **Code**: **Other names**: **Dosage & administration**: Miglustat 200 mg t.i.d. for 7 days and a single dose on Day 8, oral use, capsule **Pharma forms**: [Capsule] **Route of Admin**: [Oral use]
Placebo	Placebo Comparator	Placebo	**Name**: Matching placebo t.i.d. **Code**: **Other names**: **Dosage & administration**: Placebo t.i.d. for 7 days and a single dose on Day 8, oral use, capsule **Pharma forms**: [Capsule] **Route of Admin**: [Oral use]

Period milestone achievement

MILESTONE/REASON	MIGLUSTAT	PLACEBO
STARTED	11	11
COMPLETED	11	11
OTHER MILESTONES REASONS FOR NON-COMPLETION REASONS FOR SUBJECT JOINING		

Period 2

Period details

Period title	Overall study (Treatment 1 & 2)
Is this the baseline period	no
Allocation method	Randomised - controlled
Blinding type	Double Blind
Roles blinded	Subject, Investigator, Monitor, Data analyst
Blinding details	The investigator and study staff, the patients, the 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.
Are arms mutually exclusive	no
Subjects in period	11

Period arms

TITLE	TYPE	DESCRIPTION	PRODUCTS
Miglustat	Experimental	Miglustat 200 mg t.i.d.	**Name**: Miglustat 200 mg t.i.d. **Code**: **Other names**: **Dosage & administration**: Miglustat 200 mg t.i.d. for 7 days and a single dose on Day 8, oral use, capsule **Pharma forms**: [Capsule] **Route of Admin**: [Oral use]
Placebo	Placebo Comparator	Placebo t.i.d.	**Name**: Matching placebo t.i.d. **Code**: **Other names**: **Dosage & administration**: Placebo t.i.d. for 7 days and a single dose on Day 8, oral use, capsule **Pharma forms**: [Capsule] **Route of Admin**: [Oral use]

Period milestone achievement

MILESTONE/REASON	MIGLUSTAT	PLACEBO
STARTED	11	11
COMPLETED	11	11
OTHER MILESTONES REASONS FOR NON-COMPLETION REASONS FOR SUBJECT JOINING		

SUBJECT ANALYSIS SETS

TITLE	TYPE	DESCRIPTION	NUMBER OF SUBJECTS
All randomized set	Intention-to-treat		

		All randomized set	11
Safety set	Safety analysis	Safety set	11
Per-protocol set	Per protocol	Per-protocol set	11

BASELINE CHARACTERISTICS

Baseline characteristics information

The baseline period is	Baseline period
How are baseline characteristics reported?	Per baseline period

Reporting groups

Reporting group 1

Title	
Subjects	0
Description	Baseline period

Age characteristics

Age categorical characteristic

Characteristic title	Age Categorical
Units	Years
Description	
Categories	
Ready for collection values	no

Age continuous characteristic

Characteristic title	Age Continuous
Units	Year
Description	
Central tendency type	Arithmetic mean
Dispersion type	Standard deviation
Ready for collection values	yes

REPORTING GROUPS

GROUP#	TITLE	TENDENCY	STANDARD DEVIATION
Group 1		26.4	7.7

SUBJECT ANALYSIS SETS

SET#	TITLE	TENDENCY	STANDARD DEVIATION
Set 1	All randomized set	26.4	7.7
Set 2	Safety set		
Set 3	Per-protocol set		

Gender characteristics

Characteristic title	Gender Categorical

Units	Subjects
Description	
Categories	Female, Male
Ready for collection values	yes

REPORTING GROUPS

GROUP#	TITLE	FEMALE	MALE
Group 1		5	6
Group 2	Category totals	5	6

SUBJECT ANALYSIS SETS

SET#	TITLE	FEMALE	MALE
Set 1	All randomized set	5	6
Set 2	Safety set		
Set 3	Per-protocol set		

Study specific characteristics

END POINTS

End point 1

End point details

End point title	Change from baseline to end-of-treatment in TCS
Description	Total chloride secretion (TCS): defined as the sum of nasal potential difference (NPD) responses after perfusion with isoproterenol and chloride-free buffer in the presence of amiloride.
Timeframe	Schedule for nasal potential difference (NPD) assessments: Visit 2 and Visit 4 of Period 1, and Visit 5 and Visit 7/EOS of Period 2
End point type	Primary
Countable/Measurable	Measurable
Measurable units	mV
Measure type	Arithmetic mean
Precision/Dispersion type	Standard deviation
Reporting groups	Overall study (Treatment 1 & 2) :Miglustat, Overall study (Treatment 1 & 2) :Placebo
Subject analysis sets	Per-protocol set
Categories	

REPORTING GROUPS

PERIOD#	TITLE	SUBJECTS	MEASURE	STANDARD DEVIATION
Overall study (Treatment 1 & 2)	Miglustat	11	0.55	4.547
Overall study (Treatment 1 & 2)	Placebo	11	-0.09	3.833

SUBJECT ANALYSIS SETS

SET#	TITLE	SUBJECTS	MEASURE	STANDARD DEVIATION
Set 1	Per-protocol set	0 **Comments:** Not		

applicable

Statistical analysis 1

Statistical analysis overview

Statistical analysis title	Diff. in TCS change from baseline betw. treatments
Analysis description	Paired difference (miglustat - placebo)
Analysis type	Other
Analysis specification	Pre-specified
Arm comparison groups	Miglustat, Placebo
Subject analysis comparison groups	Per-protocol set
Primary analysis	no
Analysis type comment	LOWER LIMIT should be -3.56

Statistical test of hypothesis

P-value	0.7426
Value equality relation	=
P-value comment	UNADJUSTED TREATMENT EFFECT
Analysis method	paired t-test

Method of estimation

Confidence interval	
Number of sides	2-sided
Lower limit	3.56
Upper limit	4.83
Point estimate	
Effect estimate	0.64
Parameter	Mean difference (final values)
Dispersion type	Standard deviation
Dispersion value	6.249

End point 2

End point details

End point title	Basal NPD change from baseline (mV)
Description	
Timeframe	Schedule for nasal potential difference (NPD) assessments: Visit 2 and Visit 4 of Period 1, and Visit 5 and Visit 7/EOS of Period 2
End point type	Secondary
Countable/Measurable	Measurable
Measurable units	mV
Measure type	Arithmetic mean
Precision/Dispersion type	Standard deviation
Reporting groups	Overall study (Treatment 1 & 2) :Miglustat, Overall study (Treatment 1 & 2) :Placebo

Subject analysis sets	Per-protocol set
Categories	

REPORTING GROUPS

PERIOD#	TITLE	SUBJECTS	MEASURE	STANDARD DEVIATION
Overall study (Treatment 1 & 2)	Miglustat	11	2.18	7.054
Overall study (Treatment 1 & 2)	Placebo	11	-1.45	6.548

SUBJECT ANALYSIS SETS

SET#	TITLE	SUBJECTS	MEASURE	STANDARD DEVIATION
Set 1	Per-protocol set	0 **Comments:** Not applicable		

Statistical analysis 1

Statistical analysis overview

Statistical analysis title	Statistical analysis 1
Analysis description	
Analysis type	Other
Analysis specification	Pre-specified
Arm comparison groups	Miglustat, Placebo
Subject analysis comparison groups	Per-protocol set
Primary analysis	no
Analysis type comment	

Statistical test of hypothesis

P-value	0.1807
Value equality relation	=
P-value comment	
Analysis method	paired t-test

Method of estimation

Confidence interval	
Number of sides	
Lower limit	
Upper limit	
Point estimate	
Effect estimate	
Parameter	Not applicable
Dispersion type	
Dispersion value	

End point 3

End point details

End point title	Amiloride NPD change from baseline
Description	
Timeframe	Schedule for nasal potential difference (NPD) assessments: Visit 2 and Visit 4 of Period 1, and Visit 5 and Visit 7/EOS of Period 2
End point type	Secondary
Countable/Measurable	Measurable
Measurable units	mV
Measure type	Arithmetic mean
Precision/Dispersion type	Standard deviation
Reporting groups	Overall study (Treatment 1 & 2) :Miglustat, Overall study (Treatment 1 & 2) :Placebo
Subject analysis sets	Per-protocol set
Categories	

REPORTING GROUPS

PERIOD#	TITLE	SUBJECTS	MEASURE	STANDARD DEVIATION
Overall study (Treatment 1 & 2)	Miglustat	11	-1	10.459
Overall study (Treatment 1 & 2)	Placebo	11	-2	8.27

SUBJECT ANALYSIS SETS

SET#	TITLE	SUBJECTS	MEASURE	STANDARD DEVIATION
Set 1	Per-protocol set	0 **Comments:** Not applicable		

Statistical analysis 1

Statistical analysis overview

Statistical analysis title	Statistical analysis 1
Analysis description	
Analysis type	Other
Analysis specification	Pre-specified
Arm comparison groups	Miglustat, Placebo
Subject analysis comparison groups	Per-protocol set
Primary analysis	no
Analysis type comment	

Statistical test of hypothesis

P-value	0.7353
Value equality relation	=
P-value comment	
Analysis method	paired t-test

Method of estimation

Confidence interval

Number of sides

Lower limit

Upper limit

Point estimate

Effect estimate

Parameter

Dispersion type

Dispersion value

End point 4

End point details

End point title	Chloride-free buffer-diffusion change
Description	
Timeframe	Schedule for sweat chloride assessments: Visit 2 and Visit 4 of Period 1, and Visit 5 and Visit 7/EOS of Period 2
End point type	Secondary
Countable/Measurable	Measurable
Measurable units	mV
Measure type	Arithmetic mean
Precision/Dispersion type	Standard deviation
Reporting groups	Overall study (Treatment 1 & 2) :Miglustat, Overall study (Treatment 1 & 2) :Placebo
Subject analysis sets	Per-protocol set
Categories	

REPORTING GROUPS

PERIOD#	TITLE	SUBJECTS	MEASURE	STANDARD DEVIATION
Overall study (Treatment 1 & 2)	Miglustat	11	-1.45	4.321
Overall study (Treatment 1 & 2)	Placebo	11	0.82	3.157

SUBJECT ANALYSIS SETS

SET#	TITLE	SUBJECTS	MEASURE	STANDARD DEVIATION
Set 1	Per-protocol set	0 **Comments:** Not applicable		

Statistical analysis 1

Statistical analysis overview

Statistical analysis title	Statistical analysis 1
Analysis description	
Analysis type	Other
Analysis specification	Pre-specified
Arm comparison groups	Miglustat, Placebo

Subject analysis comparison groups	Per-protocol set
Primary analysis	no
Analysis type comment	

Statistical test of hypothesis

P-value	0.2643
Value equality relation	=
P-value comment	
Analysis method	paired t-test

Method of estimation

Confidence interval	
Number of sides	
Lower limit	
Upper limit	
Point estimate	
Effect estimate	
Parameter	
Dispersion type	
Dispersion value	

End point 5

End point details

End point title	ATP hyperpolarization change
Description	
Timeframe	Schedule for nasal potential difference (NPD) assessments: Visit 2 and Visit 4 of Period 1, and Visit 5 and Visit 7/EOS of Period 2
End point type	Secondary
Countable/Measurable	Measurable
Measurable units	mV
Measure type	Arithmetic mean
Precision/Dispersion type	Standard deviation
Reporting groups	Overall study (Treatment 1 & 2) :Miglustat, Overall study (Treatment 1 & 2) :Placebo
Subject analysis sets	Per-protocol set
Categories	

REPORTING GROUPS

PERIOD#	TITLE	SUBJECTS	MEASURE	STANDARD DEVIATION
Overall study (Treatment 1 & 2)	Miglustat	11	2.36	10.893
Overall study (Treatment 1 & 2)	Placebo	11	-2.27	16.805

SUBJECT ANALYSIS SETS

SET#	TITLE	SUBJECTS	MEASURE	STANDARD DEVIATION
Set 1	Per-protocol set	0 **Comments:** Not applicable		

Statistical analysis 1

Statistical analysis overview

Statistical analysis title	Statistical analysis 1
Analysis description	
Analysis type	Other
Analysis specification	Pre-specified
Arm comparison groups	Miglustat, Placebo
Subject analysis comparison groups	Per-protocol set
Primary analysis	no
Analysis type comment	

Statistical test of hypothesis

P-value	0.2457
Value equality relation	=
P-value comment	
Analysis method	paired t-test

Method of estimation

Confidence interval	
Number of sides	
Lower limit	
Upper limit	
Point estimate	
Effect estimate	
Parameter	
Dispersion type	
Dispersion value	

End point 6

End point details

End point title	NPD Clancy Index change
Description	
Timeframe	Schedule for nasal potential difference (NPD) assessments: Visit 2 and Visit 4 of Period 1, and Visit 5 and Visit 7/EOS of Period 2
End point type	Secondary
Countable/Measurable	Measurable
Measurable units	mV
Measure type	Arithmetic mean
Precision/Dispersion type	Standard deviation

Reporting groups	Overall study (Treatment 1 & 2) :Miglustat, Overall study (Treatment 1 & 2) :Placebo
Subject analysis sets	Per-protocol set
Categories	

REPORTING GROUPS

PERIOD#	TITLE	SUBJECTS	MEASURE	STANDARD DEVIATION
Overall study (Treatment 1 & 2)	Miglustat	11	-0.45	13.314
Overall study (Treatment 1 & 2)	Placebo	11	-2.09	8.408

SUBJECT ANALYSIS SETS

SET#	TITLE	SUBJECTS	MEASURE	STANDARD DEVIATION
Set 1	Per-protocol set	0 **Comments:** Not applicable		

Statistical analysis 1

Statistical analysis overview

Statistical analysis title	Statistical analysis 1
Analysis description	
Analysis type	Other
Analysis specification	Pre-specified
Arm comparison groups	Miglustat, Placebo
Subject analysis comparison groups	Per-protocol set
Primary analysis	no
Analysis type comment	

Statistical test of hypothesis

P-value	0.6572
Value equality relation	=
P-value comment	
Analysis method	paired t-test

Method of estimation

Confidence interval	
Number of sides	
Lower limit	
Upper limit	
Point estimate	
Effect estimate	
Parameter	
Dispersion type	
Dispersion value	

End point 7

End point details

End point title	NPD Wilschanski Index change
Description	
Timeframe	Schedule for nasal potential difference (NPD) assessments: Visit 2 and Visit 4 of Period 1, and Visit 5 and Visit 7/EOS of Period 2
End point type	Secondary
Countable/Measurable	Measurable
Measurable units	Percent
Measure type	Arithmetic mean
Precision/Dispersion type	Standard deviation
Reporting groups	Overall study (Treatment 1 & 2) :Miglustat, Overall study (Treatment 1 & 2) :Placebo
Subject analysis sets	Per-protocol set
Categories	

REPORTING GROUPS

PERIOD#	TITLE	SUBJECTS	MEASURE	STANDARD DEVIATION
Overall study (Treatment 1 & 2)	Miglustat	11	3.14	43.466
Overall study (Treatment 1 & 2)	Placebo	11	1.61	14.026

SUBJECT ANALYSIS SETS

SET#	TITLE	SUBJECTS	MEASURE	STANDARD DEVIATION
Set 1	Per-protocol set	0 **Comments:** Not applicable		

Statistical analysis 1

Statistical analysis overview

Statistical analysis title	Statistical analysis 1
Analysis description	
Analysis type	Other
Analysis specification	Pre-specified
Arm comparison groups	Miglustat, Placebo
Subject analysis comparison groups	Per-protocol set
Primary analysis	no
Analysis type comment	

Statistical test of hypothesis

P-value	0.9116
Value equality relation	=
P-value comment	
Analysis method	paired t-test

Method of estimation

Confidence interval

Number of sides

Lower limit

Upper limit

Point estimate

Effect estimate

Parameter

Dispersion type

Dispersion value

End point 8

End point details

End point title	Sweat sodium concentration change
Description	
Timeframe	Schedule for sweat sodium assessments: Visit 2 and Visit 4 of Period 1, and Visit 5 and Visit 7/EOS of Period 2
End point type	Secondary
Countable/Measurable	Measurable
Measurable units	µmol/L
Measure type	Arithmetic mean
Precision/Dispersion type	Standard deviation
Reporting groups	Overall study (Treatment 1 & 2) :Miglustat, Overall study (Treatment 1 & 2) :Placebo
Subject analysis sets	Per-protocol set
Categories	

REPORTING GROUPS

PERIOD#	TITLE	SUBJECTS	MEASURE	STANDARD DEVIATION
Overall study (Treatment 1 & 2)	Miglustat	11	-2.5	10.608
Overall study (Treatment 1 & 2)	Placebo	11	0.13	9.561

SUBJECT ANALYSIS SETS

SET#	TITLE	SUBJECTS	MEASURE	STANDARD DEVIATION
Set 1	Per-protocol set	0 **Comments:** Not applicable		

Statistical analysis 1

Statistical analysis overview

Statistical analysis title	Statistical analysis 1
Analysis description	

Analysis type	Other
Analysis specification	Pre-specified
Arm comparison groups	Miglustat, Placebo
Subject analysis comparison groups	Per-protocol set
Primary analysis	no
Analysis type comment	

Statistical test of hypothesis

P-value	0.5054
Value equality relation	=
P-value comment	
Analysis method	paired t-test

Method of estimation

Confidence interval	
Number of sides	
Lower limit	
Upper limit	
Point estimate	
Effect estimate	
Parameter	
Dispersion type	
Dispersion value	

End point 9

End point details

End point title	Sweat chloride concentration change
Description	
Timeframe	Schedule for sweat chloride assessments: Visit 2 and Visit 4 of Period 1, and Visit 5 and Visit 7/EOS of Period 2
End point type	Secondary
Countable/Measurable	Measurable
Measurable units	µmol/L
Measure type	Arithmetic mean
Precision/Dispersion type	Standard deviation
Reporting groups	Overall study (Treatment 1 & 2) :Miglustat, Overall study (Treatment 1 & 2) :Placebo
Subject analysis sets	Per-protocol set
Categories	

REPORTING GROUPS

PERIOD#	TITLE	SUBJECTS	MEASURE	STANDARD DEVIATION
Overall study (Treatment 1 & 2)	Miglustat	11	-1.86	8.999
Overall study				

(Treatment 1 & 2)	Placebo	11	-1.5	8.421

SUBJECT ANALYSIS SETS

SET#	TITLE	SUBJECTS	MEASURE	STANDARD DEVIATION
Set 1	Per-protocol set	0 **Comments:** Not applicable		

Statistical analysis 1

Statistical analysis overview

Statistical analysis title	Statistical analysis 1
Analysis description	
Analysis type	Other
Analysis specification	Pre-specified
Arm comparison groups	Miglustat, Placebo
Subject analysis comparison groups	Per-protocol set
Primary analysis	no
Analysis type comment	

Statistical test of hypothesis

P-value	0.9697
Value equality relation	=
P-value comment	
Analysis method	paired t-test

Method of estimation

Confidence interval	
Number of sides	
Lower limit	
Upper limit	
Point estimate	
Effect estimate	
Parameter	
Dispersion type	
Dispersion value	

End point 10

End point details

End point title	FEV1 change from baseline
Description	
Timeframe	Schedule for FEV1 assessments: Visit 1 (Screening), Visit 2 and Visit 4 of Period 1, and Visit 5 and Visit 7/EOS of Period 2
End point type	Secondary
Countable/Measurable	Measurable
Measurable units	Percent

Measure type	Arithmetic mean
Precision/Dispersion type	Standard deviation
Reporting groups	Overall study (Treatment 1 & 2) :Miglustat, Overall study (Treatment 1 & 2) :Placebo
Subject analysis sets	Per-protocol set
Categories	

REPORTING GROUPS

PERIOD#	TITLE	SUBJECTS	MEASURE	STANDARD DEVIATION
Overall study (Treatment 1 & 2)	Miglustat	11	-1.38	4.584
Overall study (Treatment 1 & 2)	Placebo	11	-2.29	3.983

SUBJECT ANALYSIS SETS

SET#	TITLE	SUBJECTS	MEASURE	STANDARD DEVIATION
Set 1	Per-protocol set	0 **Comments:** Not applicable		

Statistical analysis 1

Statistical analysis overview

Statistical analysis title	Statistical analysis 1
Analysis description	
Analysis type	Other
Analysis specification	Pre-specified
Arm comparison groups	Miglustat, Placebo
Subject analysis comparison groups	Per-protocol set
Primary analysis	no
Analysis type comment	

Statistical test of hypothesis

P-value	0.6422
Value equality relation	=
P-value comment	
Analysis method	paired t-test

Method of estimation

Confidence interval	
Number of sides	
Lower limit	
Upper limit	
Point estimate	
Effect estimate	
Parameter	
Dispersion type	

Dispersion value

ADVERSE EVENTS

No serious adverse events have been specified.

Adverse events information

Timeframe for adverse event reporting	AE reporting at Visit 1 (Screening) and from Visit 2 to Visit 7/EOS (i..e. up to 28 days); SAE reporting at Visit 1 (Screening) and from Visit 2 to Visit 8/Follow up (i..e. up to 57 days)
Adverse event reporting additional description	
Assessment type	systematic
Frequency threshold for reporting non-serious adverse events	5
Dictionary name	MedDRA
Version	11.0

Adverse events reporting groups

Adverse event reporting group 1

Reporting group title	Miglustat
Reporting group description	Miglustat 200 mg t.i.d. for 7 days and a single dose on Day 8
Subjects exposed	11
Subjects affected by serious adverse events	0
Subjects affected by non-serious adverse events	11
Total number of deaths (all causes)	0
Total number of deaths resulting from adverse events	0

Adverse event reporting group 2

Reporting group title	Placebo
Reporting group description	Plaebo t.i.d. for 7 days and a single dose on Day 8
Subjects exposed	11
Subjects affected by serious adverse events	0
Subjects affected by non-serious adverse events	6
Total number of deaths (all causes)	0
Total number of deaths resulting from adverse events	0

Serious adverse events

Non-serious adverse events

Non-serious adverse event 1

NON-SERIOUS ADVERSE EVENT DETAILS

System organ class	Gastrointestinal disorders
Event term	Diarrhoea
Additional description	
Assessment type	systematic
Default dictionary for reporting Adverse events in these results is	MedDRA
Do you want to use a different name and version for reporting this adverse event?	no
Dictionary name	MedDRA
Version	11.0

VALUES FOR NON-SERIOUS ADVERSE EVENT PER REPORTING GROUP

Threshold for non-serious adverse event reporting is	5%

REPORTING GROUPS	SUBJECTS AFFECTED NUMBER	SUBJECTS EXPOSED NUMBER	OCCURRENCES ALL NUMBER
MIGLUSTAT	10	11	13
PLACEBO	2	11	2

Non-serious adverse event 2

NON-SERIOUS ADVERSE EVENT DETAILS

System organ class	Gastrointestinal disorders
Event term	Abdominal discomfort
Additional description	
Assessment type	systematic
Default dictionary for reporting Adverse events in these results is	MedDRA
Do you want to use a different name and version for reporting this adverse event?	no
Dictionary name	MedDRA
Version	11.0

VALUES FOR NON-SERIOUS ADVERSE EVENT PER REPORTING GROUP

Threshold for non-serious adverse event reporting is	5%

REPORTING GROUPS	SUBJECTS AFFECTED NUMBER	SUBJECTS EXPOSED NUMBER	OCCURRENCES ALL NUMBER
MIGLUSTAT	3	11	3
PLACEBO	0	11	0

Non-serious adverse event 3

NON-SERIOUS ADVERSE EVENT DETAILS

System organ class	Nervous system disorders
Event term	Headache
Additional description	
Assessment type	systematic

Default dictionary for reporting Adverse events in these results is	MedDRA
Do you want to use a different name and version for reporting this adverse event?	no
Dictionary name	MedDRA
Version	11.0

VALUES FOR NON-SERIOUS ADVERSE EVENT PER REPORTING GROUP

Threshold for non-serious adverse event reporting is	5%

REPORTING GROUPS	SUBJECTS AFFECTED NUMBER	SUBJECTS EXPOSED NUMBER	OCCURRENCES ALL NUMBER
MIGLUSTAT	3	11	3
PLACEBO	0	11	0

Non-serious adverse event 4

NON-SERIOUS ADVERSE EVENT DETAILS

System organ class	Gastrointestinal disorders
Event term	Abdominal pain
Additional description	
Assessment type	systematic
Default dictionary for reporting Adverse events in these results is	MedDRA
Do you want to use a different name and version for reporting this adverse event?	no
Dictionary name	MedDRA
Version	11.0

VALUES FOR NON-SERIOUS ADVERSE EVENT PER REPORTING GROUP

Threshold for non-serious adverse event reporting is	5%

REPORTING GROUPS	SUBJECTS AFFECTED NUMBER	SUBJECTS EXPOSED NUMBER	OCCURRENCES ALL NUMBER
MIGLUSTAT	2	11	2
PLACEBO	2	11	2

Non-serious adverse event 5

NON-SERIOUS ADVERSE EVENT DETAILS

System organ class	Respiratory, thoracic and mediastinal disorders
Event term	Pharyngolaryngeal pain
Additional description	
Assessment type	systematic
Default dictionary for reporting Adverse events in these results is	MedDRA
Do you want to use a different name and version for reporting this adverse event?	no
Dictionary name	MedDRA
Version	11.0

VALUES FOR NON-SERIOUS ADVERSE EVENT PER REPORTING GROUP

Threshold for non-serious adverse event reporting is	5%

REPORTING GROUPS	SUBJECTS AFFECTED NUMBER	SUBJECTS EXPOSED NUMBER	OCCURRENCES ALL NUMBER
MIGLUSTAT	2	11	2
PLACEBO	0	11	0

Non-serious adverse event 6

NON-SERIOUS ADVERSE EVENT DETAILS

System organ class	Gastrointestinal disorders
Event term	Aphthous stomatitis
Additional description	
Assessment type	systematic
Default dictionary for reporting Adverse events in these results is	MedDRA
Do you want to use a different name and version for reporting this adverse event?	no
Dictionary name	MedDRA
Version	11.0

VALUES FOR NON-SERIOUS ADVERSE EVENT PER REPORTING GROUP

Threshold for non-serious adverse event reporting is	5%

REPORTING GROUPS	SUBJECTS AFFECTED NUMBER	SUBJECTS EXPOSED NUMBER	OCCURRENCES ALL NUMBER
MIGLUSTAT	1	11	1
PLACEBO	0	11	0

Non-serious adverse event 7

NON-SERIOUS ADVERSE EVENT DETAILS

System organ class	General disorders and administration site conditions
Event term	Chills
Additional description	
Assessment type	systematic
Default dictionary for reporting Adverse events in these results is	MedDRA
Do you want to use a different name and version for reporting this adverse event?	no
Dictionary name	MedDRA
Version	11.0

VALUES FOR NON-SERIOUS ADVERSE EVENT PER REPORTING GROUP

Threshold for non-serious adverse event reporting is	5%

REPORTING GROUPS	SUBJECTS AFFECTED NUMBER	SUBJECTS EXPOSED NUMBER	OCCURRENCES ALL NUMBER
MIGLUSTAT	1	11	1

PLACEBO	0	11	0

Non-serious adverse event 8

NON-SERIOUS ADVERSE EVENT DETAILS

System organ class	Nervous system disorders
Event term	Dizziness
Additional description	
Assessment type	systematic
Default dictionary for reporting Adverse events in these results is	MedDRA
Do you want to use a different name and version for reporting this adverse event?	no
Dictionary name	MedDRA
Version	11.0

VALUES FOR NON-SERIOUS ADVERSE EVENT PER REPORTING GROUP

Threshold for non-serious adverse event reporting is	5%

REPORTING GROUPS	SUBJECTS AFFECTED NUMBER	SUBJECTS EXPOSED NUMBER	OCCURRENCES ALL NUMBER
MIGLUSTAT	1	11	1
PLACEBO	0	11	0

Non-serious adverse event 9

NON-SERIOUS ADVERSE EVENT DETAILS

System organ class	Gastrointestinal disorders
Event term	Gastrointestinal disorder
Additional description	
Assessment type	systematic
Default dictionary for reporting Adverse events in these results is	MedDRA
Do you want to use a different name and version for reporting this adverse event?	no
Dictionary name	MedDRA
Version	11.0

VALUES FOR NON-SERIOUS ADVERSE EVENT PER REPORTING GROUP

Threshold for non-serious adverse event reporting is	5%

REPORTING GROUPS	SUBJECTS AFFECTED NUMBER	SUBJECTS EXPOSED NUMBER	OCCURRENCES ALL NUMBER
MIGLUSTAT	1	11	1
PLACEBO	0	11	0

Non-serious adverse event 10

NON-SERIOUS ADVERSE EVENT DETAILS

System organ class	Vascular disorders

Event term	Hypertension
Additional description	
Assessment type	systematic
Default dictionary for reporting Adverse events in these results is	MedDRA
Do you want to use a different name and version for reporting this adverse event?	no
Dictionary name	MedDRA
Version	11.0

VALUES FOR NON-SERIOUS ADVERSE EVENT PER REPORTING GROUP

Threshold for non-serious adverse event reporting is	5%

REPORTING GROUPS	SUBJECTS AFFECTED NUMBER	SUBJECTS EXPOSED NUMBER	OCCURRENCES ALL NUMBER
MIGLUSTAT	1	11	1
PLACEBO	0	11	0

Non-serious adverse event 11

NON-SERIOUS ADVERSE EVENT DETAILS

System organ class	Psychiatric disorders
Event term	Insomnia
Additional description	
Assessment type	systematic
Default dictionary for reporting Adverse events in these results is	MedDRA
Do you want to use a different name and version for reporting this adverse event?	no
Dictionary name	MedDRA
Version	11.0

VALUES FOR NON-SERIOUS ADVERSE EVENT PER REPORTING GROUP

Threshold for non-serious adverse event reporting is	5%

REPORTING GROUPS	SUBJECTS AFFECTED NUMBER	SUBJECTS EXPOSED NUMBER	OCCURRENCES ALL NUMBER
MIGLUSTAT	1	11	1
PLACEBO	0	11	0

Non-serious adverse event 12

NON-SERIOUS ADVERSE EVENT DETAILS

System organ class	Infections and infestations
Event term	Pseudomonas infection
Additional description	
Assessment type	systematic
Default dictionary for reporting Adverse events in these results is	MedDRA
Do you want to use a different name	

and version for reporting this adverse event?	no
Dictionary name	MedDRA
Version	11.0

VALUES FOR NON-SERIOUS ADVERSE EVENT PER REPORTING GROUP

Threshold for non-serious adverse event reporting is	5%

REPORTING GROUPS	SUBJECTS AFFECTED NUMBER	SUBJECTS EXPOSED NUMBER	OCCURRENCES ALL NUMBER
MIGLUSTAT	1	11	1
PLACEBO	0	11	0

Non-serious adverse event 13

NON-SERIOUS ADVERSE EVENT DETAILS

System organ class	Gastrointestinal disorders
Event term	Stomach discomfort
Additional description	
Assessment type	systematic
Default dictionary for reporting Adverse events in these results is	MedDRA
Do you want to use a different name and version for reporting this adverse event?	no
Dictionary name	MedDRA
Version	11.0

VALUES FOR NON-SERIOUS ADVERSE EVENT PER REPORTING GROUP

Threshold for non-serious adverse event reporting is	5%

REPORTING GROUPS	SUBJECTS AFFECTED NUMBER	SUBJECTS EXPOSED NUMBER	OCCURRENCES ALL NUMBER
MIGLUSTAT	1	11	1
PLACEBO	0	11	0

Non-serious adverse event 14

NON-SERIOUS ADVERSE EVENT DETAILS

System organ class	Metabolism and nutrition disorders
Event term	Decreased appetite
Additional description	
Assessment type	systematic
Default dictionary for reporting Adverse events in these results is	MedDRA
Do you want to use a different name and version for reporting this adverse event?	no
Dictionary name	MedDRA
Version	11.0

VALUES FOR NON-SERIOUS ADVERSE EVENT PER REPORTING GROUP

Threshold for non-serious adverse event reporting is	5%

REPORTING GROUPS	SUBJECTS AFFECTED NUMBER	SUBJECTS EXPOSED NUMBER	OCCURRENCES ALL NUMBER
MIGLUSTAT	0	11	0
PLACEBO	1	11	1

Non-serious adverse event 15

NON-SERIOUS ADVERSE EVENT DETAILS

System organ class	Respiratory, thoracic and mediastinal disorders
Event term	Epistaxis
Additional description	
Assessment type	systematic
Default dictionary for reporting Adverse events in these results is	MedDRA
Do you want to use a different name and version for reporting this adverse event?	no
Dictionary name	MedDRA
Version	11.0

VALUES FOR NON-SERIOUS ADVERSE EVENT PER REPORTING GROUP

Threshold for non-serious adverse event reporting is	5%

REPORTING GROUPS	SUBJECTS AFFECTED NUMBER	SUBJECTS EXPOSED NUMBER	OCCURRENCES ALL NUMBER
MIGLUSTAT	0	11	0
PLACEBO	1	11	1

Non-serious adverse event 16

NON-SERIOUS ADVERSE EVENT DETAILS

System organ class	Psychiatric disorders
Event term	Initial insomnia
Additional description	
Assessment type	systematic
Default dictionary for reporting Adverse events in these results is	MedDRA
Do you want to use a different name and version for reporting this adverse event?	no
Dictionary name	MedDRA
Version	11.0

VALUES FOR NON-SERIOUS ADVERSE EVENT PER REPORTING GROUP

Threshold for non-serious adverse event reporting is	5%

REPORTING GROUPS	SUBJECTS AFFECTED NUMBER	SUBJECTS EXPOSED NUMBER	OCCURRENCES ALL NUMBER
MIGLUSTAT	0	11	0
PLACEBO	1	11	1

Non-serious adverse event 17

NON-SERIOUS ADVERSE EVENT DETAILS

System organ class	Respiratory, thoracic and mediastinal disorders
Event term	Rhinorrhoea
Additional description	
Assessment type	systematic
Default dictionary for reporting Adverse events in these results is	MedDRA
Do you want to use a different name and version for reporting this adverse event?	no
Dictionary name	MedDRA
Version	11.0

VALUES FOR NON-SERIOUS ADVERSE EVENT PER REPORTING GROUP

Threshold for non-serious adverse event reporting is	5%

REPORTING GROUPS	SUBJECTS AFFECTED NUMBER	SUBJECTS EXPOSED NUMBER	OCCURRENCES ALL NUMBER
MIGLUSTAT	0	11	0
PLACEBO	1	11	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

Limitations and caveats applicable to this summary of the results	The proportion of measurements with a residual TCS response (≤ -5 mV) was unexpectedly high (>60%), jeopardizing any meaningful interpretation of the primary endpoint. NPD measurements were highly variable, probably due to methodological aspects.

Online references

http://www.ncbi.nlm.nih.gov/pubmed/22281182