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A phase I/II randomised control study of OGT 918 in patients with Niemann-Pick type C disease

Submission date	Recruitment status	[ ] Prospectively registered
05/07/2007	No longer recruiting	[ ] Protocol
Registration date	**Overall study status**	[ ] Statistical analysis plan
26/07/2007	Completed	[X] Results
Last Edited	**Condition category**	[ ] Individual participant data
02/10/2014	Nervous System Diseases	

Plain English summary of protocol
Not provided at time of registration

Contact information

Type(s)
Scientific

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Additional identifiers

EudraCT/CTIS number

IRAS number

ClinicalTrials.gov number
NCT00517153

Secondary identifying numbers
OGT 918-007

Study information

Scientific Title

Study objectives
Niemann-Pick type C disease is an inherited neurodegenerative disorder characterised by an intracellular lipid-trafficking defect with secondary accumulation of glycosphingolipids (GSLs).

The purpose of the study was to evaluate the effects of miglustat (OGT 918) as a treatment for Niemann-Pick type C disease in adult, juvenile and paediatric patients over a 24-month treatment period. We hypothesised that patients in the treatment group would show slower rates of decline or stabilisation in one or more markers of the disease compared to the standard care group.

The study was initially supported by Oxford GlycoSciences, the original manufacturer of miglustat (OGT 918). During the study the sponsor changed from Oxford GlycoSciences, a wholly-owned subsidiary of Celltech R&D Ltd, to Actelion Pharmaceuticals Ltd.

Ethics approval required
Old ethics approval format

Ethics approval(s)
1. Centre 1: Salford and Trafford LREC, 24/12/2001, ref: 01266
2. Centre 2: Institutional Review Board of Columbia Presbyterian Medical Centre, 05/04/2002, ref: 14413

Study design
Randomised controlled intervention study conducted at two centres

Primary study design
Interventional

Secondary study design
Randomised controlled trial

Study setting(s)
Not specified

Study type(s)
Treatment

Participant information sheet

Health condition(s) or problem(s) studied
Niemann-Pick type C disease

Interventions
Patients in the juvenile/adult group (12 years or older) were randomised in a 2:1 ratio to either miglustat 200 mg three times daily orally (p.o.) for 12 months or standard symptomatic care (no study drug) as a control group.

Both miglustat-treated and standard care groups received other concomitant medications for standard indications throughout the study. All children received miglustat in a dose adjusted according to Body Surface Area (BSA).

Patients were assessed one week after commencing miglustat therapy and monthly thereafter with dose modification as clinically indicated.

Intervention Type
Drug

Phase
Phase I/II

Drug/device/biological/vaccine name(s)
Miglustat (OGT 918)

Primary outcome measure
Primary efficacy endpoint: change from baseline in Horizontal Saccadic Eye Movement (HSEM)-alpha (a measure of HSEM velocity) at 12 months or last available value

Secondary outcome measures
Secondary efficacy endpoints:
1. HSEM-beta
2. Assessments of swallowing (at screening and months 6 and 12; the assessor evaluated the patient's swallowing ability with prespecified substances, using a five-degree category scale from 'no problems of swallowing' to 'could not swallow the substance at all')
3. Auditory acuity (part of neurological examination at screening and months 3, 6, 9 and 12)
4. Ambulatory ability (standard ambulation index; part of neurological examination at screening and months 3, 6, 9 and 12)
5. Cognition (Mini Mental Status Examination [MMSE]; at screening and months 3, 6, 9, 12)

Overall study start date
01/03/2003

Completion date
30/04/2004

Eligibility

Key inclusion criteria
1. Juveniles and adults (12 years and over) and paediatric patients aged 4 - 11 years
2. Patients with Niemann-Pick type C disease confirmed by reduced cholesterol esterification and abnormal filipin staining in cultured fibroblasts
3. Capable of cooperating with physical examination and other testing

Participant type(s)
Patient

Age group
Other

Sex
Both

Target number of participants
41

Key exclusion criteria
1. Clinically significant diarrhoea (greater than three liquid stools per day for more than 7 days without definable cause) within 3 months before enrolment
2. Significant gastrointestinal disorders or other intercurrent illnesses

Date of first enrolment
01/03/2003

Date of final enrolment
30/04/2004

Locations

Countries of recruitment
United Kingdom

United States of America

Study participating centre
Division of Pediatric Neurology
New York
United States of America
NY 10032

Sponsor information

Organisation
Actelion Pharmaceuticals Ltd (Switzerland)

Sponsor details
c/o Dr Cynthia Calabresse
Gewerbestrasse 16
Allschwil

Switzerland
4123

Sponsor type
Industry

Website
http://www.actelion.com/

ROR
https://ror.org/001yedb91

Funder(s)

Funder type
Industry

Funder Name
Actelion Pharmaceuticals Ltd (Switzerland)

Results and Publications

Publication and dissemination plan
Not provided at time of registration

Intention to publish date

Individual participant data (IPD) sharing plan

IPD sharing plan summary
Not provided at time of registration

Study outputs

Output type	Details	Date created	Date added	Peer reviewed?	Patient-facing?
Results article	results	01/09/2007		Yes	No