An Open-Label, Multicenter, Extension Study to Evaluate the Long-Term Safety and Efficacy of Dexpramipexole (BIIB050) in Subjects With Amyotrophic Lateral Sclerosis

Published: 14-06-2012
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Primary:The primary objective of the study is to evaluate the long-term safety profile of dexpramipexole in subjects with ALS.Secondary:The secondary objective of this study is to evaluate the longterm efficacy of dexpramipexole in this study...

Ethical review	Approved WMO
Status	Will not start
Health condition type	Neuromuscular disorders
Study type	Interventional

Summary

ID

NL-OMON37292

Source
ToetsingOnline

Brief title
ENVISION

Condition

- Neuromuscular disorders

Synonym
Amyotrophic Lateral Sclerosis, Lou Gehrig's disease

Research involving
Human

Sponsors and support

Primary sponsor: Biogen Idec Limited
Source(s) of monetary or material Support: the pharmaceutical industry.

Intervention

Keyword: ALS, Amyotrophic Lateral Sclerosis, Long term, Motor Neurone Disease

Outcome measures

Primary outcome

Primary:

The following primary endpoints will be evaluated:

• incidence of adverse events (AEs) and serious adverse events (SAEs)

• discontinuation of study treatment due to an AE

• changes in vital signs, clinical laboratory assessments (hematology, blood chemistry, and urinalysis), 12-lead electrocardiograms (ECGs) and body weight

• incidence of laboratory abnormalities

Secondary outcome

Secondary:

The following secondary endpoints will be evaluated:

• change in ALS Functional Rating Scale-Revised (ALSFRS-R) score

• decline in sniff nasal inspiratory pressure (SNIP)

• time to death

• time to death or death equivalent (tracheostomy or permanent assisted ventilation [PAV], defined as use of noninvasive ventilation [NIV] for >= 22 hours per day for >= 10 days

Study description

Background summary

Currently, only 1 medicine, Rilutek® (riluzole, Sanofi Aventis, approved by the United States [US] Food and Drug Administration in 1995 and the European Medicines Agency in 1996), is available for the treatment of ALS. Considering the seriousness of the disease, the lack of robust efficacy of riluzole, and limited options for further treatment, there remains a pressing unmet medical need for effective and safe treatments for ALS. In a 2-part Phase 2 study conducted by Knopp Neurosciences Inc. (KNS 760704-CL201 [CL201]), dexpramipexole appeared to be well tolerated at doses ranging from 25 mg to 150 mg twice daily. Study results also showed a dose-dependent trend in slowing the rate of functional decline, as measured by change in the ALS Functional Rating Scale-Revised (ALSFRS-R), and a trend toward reduction in mortality of the 150 mg twice daily group compared to the 25 mg twice daily group.
This extension study will further evaluate the long-term safety and efficacy profile of dexpramipexole.

Study objective

Primary:
The primary objective of the study is to evaluate the long-term safety profile of dexpramipexole in subjects with ALS.

Secondary:
The secondary objective of this study is to evaluate the longterm efficacy of dexpramipexole in this study population using clinical endpoints measuring function and survival.

Study design

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Intervention

All subjects will receive 300 mg dexpramipexole per day (150 mg given twice daily).

Study burden and risks

After taking the study medication the patient will be asked to return to the clinic on the following times:

• at Month 1, Month 2, Month 4, and Month 6
• once every 4 months from Month 10 through Month 34 (at Months 10, 14, 18, 22, 26, 30, and 34)
• at Month 36 (or within 7 days after your last dose of study medicine if you stop study medicine before the end of the study)
• 1 month after your last dose of study medicine.

Every other month, the research personnel will conduct evaluations by calling you and/or visiting the patients at home. The patients will be contacted by the study staff at Months 3 and 5, and every 4 months from Month 8 through Month 32 (at Months 8, 12, 16, 20, 24, 28, and 32).

• Medical history update and physical examination
• Blood pressure, heart rate, respiration rate, temperature, weight, and height
• Blood tests
• Urine tests
• Pregnancy tests (if applicable)
• Blood tests to check the level of study medicine in your blood (only if you have a serious side effect)
• Electrocardiogram (ECG)
• Lung function tests
• Questions about difficulties you may have had, how well you feel, and how well you are able to do your usual activities
• Questions about if you are taking the study medicine as instructed
• Questions about any other treatments you are taking
• Questions about if you have any new side effects

Contacts

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Scientific
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Trial sites

Listed location countries

Netherlands

Eligibility criteria

Age
Adults (18-64 years)
Elderly (65 years and older)

Inclusion criteria

1. Subject has the ability to understand the purpose and risks of the study and provide signed and dated informed consent (or have the consent confirmed by a witness if unable to write) and authorization to use protected health information (PHI) in accordance with national and local subject privacy regulations.;2. Subject was enrolled in either Study CL211 or Study 223AS302.;3. Subject has completed their last visit in Study CL211 or Study 223AS302.;4. Subjects of childbearing potential must practice effective contraception during the study and be willing and able to continue contraception for 1 month (females) or 3 months (males) after their last dose of study treatment.

Exclusion criteria

1. Subject withdrew prematurely from Study CL211 or Study 223AS302.;2. Subject permanently discontinued study treatment in Study CL211 or Study 223AS302 for any reason other than enrollment into this study.;3. Subject from Study CL211 or Study 223AS302 has a significant change in medical history (including laboratory tests or a clinically significant condition) that in the opinion of the Investigator would impair the subject*s medical fitness for participation and preclude treatment.;4. Female subject who is pregnant or breastfeeding.;5. Subject is currently enrolled in any investigational drug study other than Study CL211 or Study 223AS302.;6. Subject is taking pramipexole, other dopamine agonists, any other agent with dopaminergic activity, or any other disallowed concomitant medication;7. Subject is unwilling or unable to comply with the requirements of the protocol including the presence of any condition (physical, mental, or social) that is likely to affect the subject*s ability to comply with the protocol. At a minimum, subjects who are not able to travel to the study site must be willing to agree to remote blood draws for clinical laboratory evaluations and telephone visits to report AEs, concomitant medications, and ALSFRS-R scores.

Study design

Design

Study phase:	3
Study type:	Interventional
Masking:	Open (masking not used)
Control:	Uncontrolled
Primary purpose:	Treatment

Recruitment

NL

Recruitment status:	Will not start
Start date (anticipated):	30-09-2012
Enrollment:	37
Type:	Anticipated

Medical products/devices used

Product type:	Medicine
Brand name:	n/a
Generic name:	Dexpramipexole

Ethics review

Approved WMO Date:	14-06-2012
Application type:	First submission
Review commission:	METC Universitair Medisch Centrum Utrecht (Utrecht)
Approved WMO Date:	30-08-2012
Application type:	First submission
Review commission:	METC Universitair Medisch Centrum Utrecht (Utrecht)
Approved WMO Date:	12-12-2012
Application type:	Amendment

Review commission: METC Universitair Medisch Centrum Utrecht (Utrecht)

Study registrations

Followed up by the following (possibly more current) registration

No registrations found.

Other (possibly less up-to-date) registrations in this register

No registrations found.

In other registers

Register	ID
EudraCT	EUCTR2011-006119-70-NL
CCMO	NL40561.041.12