A Multicenter, Open-Label, Extension Study to Evaluate the Long-term Safety and Efficacy of Patisiran in Patients with Familial Amyloidotic Polyneuropathy Who Have Completed a Prior Clinical Study with Patisiran

Published: 14-03-2016
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To assess the safety and efficacy of long-term dosing with patisiran in transthyretin-mediated amyloidosis (ATTR) patients with familial amyloidotic polyneuropathy (FAP)

Ethical review	Approved WMO
Status	Completed
Health condition type	Neurological disorders congenital
Study type	Interventional

Summary

ID

NL-OMON50597

Source
ToetsingOnline

Brief title
TTR206

Condition

- Neurological disorders congenital
- Neurological disorders NEC

Synonym
Familial ATTR, FAP (familial amyloidotic polyneuropathy)

Research involving

Human

Sponsors and support

Primary sponsor: Alnylam Pharmaceuticals
Source(s) of monetary or material Support: Alnylam Pharmaceuticals

Intervention

Keyword: ALN-TTR02, Familial amyloidotic polyneuropathy, patisiran, RNAi therapeutic

Outcome measures

Primary outcome

The objective of this study is to assess the safety and efficacy of long-term

dosing with patisiran in ATTR patients with FAP.

Secondary outcome

None

Study description

Background summary

This is a multicenter, open-label extension study designed to evaluate the long-term safety and efficacy of patisiran in patients with FAP who have completed a prior study with patisiran.

Study objective

To assess the safety and efficacy of long-term dosing with patisiran in transthyretin-mediated amyloidosis (ATTR) patients with familial amyloidotic polyneuropathy (FAP)

Study design

This is a multicenter, multinational, open-label extension study to evaluate the long-term safety and efficacy of patisiran in patients with FAP who have previously completed a patisiran study.

Intervention

Patients will receive 0.3 mg/kg patisiran once every 21 days administered as an IV infusion over 80 minutes (approximately 1ml/min for the first 15 minutes followed by approximately 3 ml/min for the remaining part of the infusion) by a controlled infusion device.

Study burden and risks

Patients are required to visit the hospital more often than during standard treatment. The patient's participation in this study will last for approximately 5 years. Efficacy and safety testing will be performed. Visits involve the administration of study medication and standard safety tests

Contacts

Public
Alnylam Pharmaceuticals

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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. Male or female of 18 to 85 years of age (inclusive)
2. Have a diagnosis of FAP with documented TTR mutation
3. Have completed a patisiran study
4. Adequate liver function and renal function

Exclusion criteria

1. Pregnant or nursing
2. Have an active infection requiring systemic antiviral or antimicrobial therapy
3. Have poorly controlled diabetes mellitus
4. Have uncontrolled clinically significant cardiac arrhythmia or unstable angina

Study design

Design

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

Recruitment

NL

Recruitment status:	Completed
Start date (anticipated):	12-01-2017
Enrollment:	3
Type:	Actual

Ethics review

Approved WMO

Date:	14-03-2016
Application type:	First submission
Review commission:	CCMO: Centrale Commissie Mensgebonden Onderzoek (Den Haag)
Approved WMO Date:	17-11-2016
Application type:	First submission
Review commission:	CCMO: Centrale Commissie Mensgebonden Onderzoek (Den Haag)
Approved WMO Date:	20-12-2016
Application type:	Amendment
Review commission:	CCMO: Centrale Commissie Mensgebonden Onderzoek (Den Haag)
Approved WMO Date:	16-01-2017
Application type:	Amendment
Review commission:	CCMO: Centrale Commissie Mensgebonden Onderzoek (Den Haag)
Approved WMO Date:	01-03-2017
Application type:	Amendment
Review commission:	CCMO: Centrale Commissie Mensgebonden Onderzoek (Den Haag)
Approved WMO Date:	23-05-2017
Application type:	Amendment
Review commission:	CCMO: Centrale Commissie Mensgebonden Onderzoek (Den Haag)
Approved WMO Date:	20-07-2017
Application type:	Amendment
Review commission:	CCMO: Centrale Commissie Mensgebonden Onderzoek (Den Haag)
Approved WMO Date:	29-08-2017
Application type:	Amendment
Review commission:	CCMO: Centrale Commissie Mensgebonden Onderzoek (Den

	Haag)
Approved WMO Date:	05-03-2018
Application type:	Amendment
Review commission:	CCMO: Centrale Commissie Mensgebonden Onderzoek (Den Haag)
Approved WMO Date:	22-06-2018
Application type:	Amendment
Review commission:	CCMO: Centrale Commissie Mensgebonden Onderzoek (Den Haag)
Approved WMO Date:	19-07-2018
Application type:	Amendment
Review commission:	CCMO: Centrale Commissie Mensgebonden Onderzoek (Den Haag)
Approved WMO Date:	03-08-2018
Application type:	Amendment
Review commission:	CCMO: Centrale Commissie Mensgebonden Onderzoek (Den Haag)
Approved WMO Date:	24-08-2018
Application type:	Amendment
Review commission:	CCMO: Centrale Commissie Mensgebonden Onderzoek (Den Haag)
Approved WMO Date:	14-05-2019
Application type:	Amendment
Review commission:	CCMO: Centrale Commissie Mensgebonden Onderzoek (Den Haag)
Approved WMO Date:	07-06-2019
Application type:	Amendment
Review commission:	CCMO: Centrale Commissie Mensgebonden Onderzoek (Den Haag)
Approved WMO Date:	08-10-2019

Application type:	Amendment
Review commission:	CCMO: Centrale Commissie Mensgebonden Onderzoek (Den Haag)
Approved WMO Date:	12-11-2019
Application type:	Amendment
Review commission:	CCMO: Centrale Commissie Mensgebonden Onderzoek (Den Haag)
Approved WMO Date:	13-03-2020
Application type:	Amendment
Review commission:	CCMO: Centrale Commissie Mensgebonden Onderzoek (Den Haag)
Approved WMO Date:	16-07-2020
Application type:	Amendment
Review commission:	CCMO: Centrale Commissie Mensgebonden Onderzoek (Den Haag)
Approved WMO Date:	27-07-2020
Application type:	Amendment
Review commission:	CCMO: Centrale Commissie Mensgebonden Onderzoek (Den Haag)
Approved WMO Date:	23-10-2020
Application type:	Amendment
Review commission:	CCMO: Centrale Commissie Mensgebonden Onderzoek (Den Haag)
Approved WMO Date:	05-11-2020
Application type:	Amendment
Review commission:	CCMO: Centrale Commissie Mensgebonden Onderzoek (Den Haag)

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	EUCTR2014-003877-40-NL
ClinicalTrials.gov	NCT01960348
CCMO	NL56671.000.16

Study results

Date completed:	23-11-2022
Results posted:	21-09-2023
Actual enrolment:	2

First publication
27-06-2023