Natural History of the Progression of X-Linked Retinitis Pigmentosa (XLRP)

Published: 22-05-2018
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The objective of the study is to gain a better understanding of disease progression over time in male subjects with X-linked retinitis pigmentosa (XLRP).

Ethical review	Approved WMO
Status	Recruiting
Health condition type	Retina, choroid and vitreous haemorrhages and vascular disorders
Study type	Observational invasive

Summary

ID

NL-OMON50458

Source
ToetsingOnline

Brief title
XOLARIS study

Condition

- Retina, choroid and vitreous haemorrhages and vascular disorders

Synonym
night blindness, tunnel vision

Research involving
Human

Sponsors and support

Primary sponsor: NightstaRx Ltd.
Source(s) of monetary or material Support: Biotech Company

Intervention

Keyword: degeneration of retina cells, impairment of night vision, Natural History, X-Linked

Retinitis Pigmentosa

Outcome measures

Primary outcome

The primary endpoints of the study are

1) change from Baseline in Early Treatment Diabetic Retinopathy Study (ETDRS)

best-corrected visual acuity (BCVA) over time.

2) change from Baseline in retinal sensitivity assessed with microperimetry

over time.

Secondary outcome

The secondary endpoints are changes from Baseline in other functional and

anatomical measures over time.

Study description

Background summary

Blindness associated with any retinal disorder is extremely debilitating and significantly impacts the patient*s quality of life. XLRP is incurable and treatment is supportive at best. There are no currently marketed therapies available for modifying the progression of XLRP. A significant unmet medical need exists for new and effective therapies for XLRP, especially those designed to halt or significantly reduce the rate of progression.
Rare diseases such as XLRP are often poorly characterised due to the scarcity of data available from a limited patient population. Thus, drug development aimed at treating XLRP is hampered by insufficient knowledge of the natural history of the disease. The objective of the study is to gain a better understanding of disease progression over time in subjects with XLRP.

Study objective

The objective of the study is to gain a better understanding of disease progression over time in male subjects with X-linked retinitis pigmentosa (XLRP).

Study design

This is a multicentre, prospective, observational study consisting of 7 visits over a 24-month period:
Visit 1 (Screening/Baseline Visit);
Visit 2 (Month 3);
Visit 3 (Month 6);
Visit 4 (Month 9);
Visit 5 (Month 12);
Visit 6 (Month 18) and
Visit 7 (Month 24/End of Study or Early Termination Visit [if applicable]).

If a subject is discontinued early from the study, every reasonable effort will be made to complete the assessments scheduled for the Early Termination Visit.

The study will enroll 2 BCVA subgroups, with a minimum of 120 subjects in subgroup 2::
• subgroup 1: ETDRS BCVA >= 74 letters (Equivalent to: Snellen 6/9 or 20/32; decimal 0.63; LogMar 0.2)
• subgroup 2: ETDRS BCVA 34-73 letters, inclusive (Equivalent to: Snellen 6/12 - 6/60 or 20/40 - 20/200; decimal 0.5 - 0.1; LogMar 0.3-1.0)
Study enrollment will be actively monitored, and enrollment into the individual BCVA subgroups may be discontinued if the target sample size is reached.

Study burden and risks

This is an observational study. As no study drug is administered, there are no risks or precautions related to administration of a study drug. The only possible risks for participants are minimal and limited to the risks associated with the research procedures done that are already part of the regular examinations in the treatment of eye disease.

Contacts

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Trial sites

Listed location countries

Netherlands

Eligibility criteria

Age
Adolescents (12-15 years)
Adolescents (16-17 years)
Adults (18-64 years)
Children (2-11 years)
Elderly (65 years and older)

Inclusion criteria

a. Subject / legal guardian (if applicable) is willing and able to provide informed consent and subject assent for participation in the study.
b. Are male and >= 7 years of age.
c. Have documentation of a pathogenic mutation in the retinitis pigmentosa GTPase regulator (RPGR) gene
d. Are willing and able to undergo ophthalmic examinations, as required by protocol, for up to 24 months.
e. Have a BCVA in at least 1 eye, as defined below:
• ETDRS BCVA >=74 letters
(Equivalent to: Snellen 6/9 or 20/32; decimal 0.63; LogMAR 0.2)
• ETDRS BCVA 34-73 letters, inclusive
(Equivalent to: Snellen 6/12 - 6/60 or 20/40 - 20/200; decimal 0.5 - 0.1; LogMAR 0.3-1.0)
Eligibility by BCVA will be divided into these 2 subgroups, with a minimum of 120 eyes in the second subgroup (34-73 letters).
f. Mean total retinal sensitivity in at least 1 eye as assessed by microperimetry >=0.1 decibels (dB) and <=20 dB*
*Subjects enrolled under the previous version of the protocol are still eligible for continuation in this study irrespective of their baseline total retinal sensitivity in the study eye.

Exclusion criteria

a. Have a history of amblyopia in the eligible eye.
b. Have any other significant ocular or non-ocular disease/disorder which, in the opinion of the investigator, may put the subject at risk because of participation in the study, may influence the results of the study, may influence the subject*s ability to perform study diagnostic tests, or impact the subject*s ability to participate in the study. This includes clinically significant cataracts.
c. Have participated in another research study involving an investigational medicinal product in the past 12 weeks or received a gene/cell-based therapy at any time previously (including but not limited to Intelligent Implant System implantation, ciliary neurotrophic factor therapy, nerve growth factor therapy).

Study design

Design

Study type: Observational invasive

Masking:	Open (masking not used)
Control:	Uncontrolled
Primary purpose:	Other

Recruitment

NL

Recruitment status:	Recruiting
Start date (anticipated):	10-09-2018
Enrollment:	30
Type:	Actual

Ethics review

Approved WMO

Date:	22-05-2018
Application type:	First submission
Review commission:	CMO regio Arnhem-Nijmegen (Nijmegen)

Approved WMO Date:	29-05-2018
Application type:	Amendment
Review commission:	CMO regio Arnhem-Nijmegen (Nijmegen)
Approved WMO Date:	05-02-2019
Application type:	Amendment
Review commission:	CMO regio Arnhem-Nijmegen (Nijmegen)
Approved WMO Date:	16-09-2020
Application type:	Amendment
Review commission:	CMO regio Arnhem-Nijmegen (Nijmegen)

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
CCMO	NL63568.091.17