ISRCTN57691385 https://doi.org/10.1186/ISRCTN57691385

A follow-on study for second-eye treatment for participants previously treated with gene therapy for X-Linked Retinitis Pigmentosa (XLRP)

Submission date
06/08/2024

Recruitment status
No longer recruiting

Registration date
13/05/2025

Overall study status
Ongoing

Last Edited
18/11/2026

Condition category
Eye Diseases

[_] Prospectively registered
[_] Protocol
[_] Statistical analysis plan
[_] Results
[_] Individual participant data
[X] Record updated in last year

Plain English summary of protocol

Background and study aims
X-linked retinitis pigmentosa (XLRP) is an inherited eye condition that causes severe eye problems, eventually leading to blindness. It is commonly caused due to an alteration in a gene* called retinitis pigmentosa guanosine triphosphatase regulator (RPGR). Currently, there are no approved treatments available for XLRP.
*A part of DNA that contains the information for making a specific protein.

The study drug, AAV5-hRKp.RPGR, is a modified gene delivery system that carries a healthy version of the RPGR gene into the cells of an eye. This gene may help to improve and preserve vision. In this study, researchers want to learn how safe and tolerable it is to deliver AAV5-hRKp.RPGR under the retina into the second eye of a person who has already received it in their first eye.

Who can participate?
Male participants aged 5 years & older, previously treated with AAV5-hRKp.RPGR in one eye in the study MGT009 and have completed or are currently enrolled in Study MGT010.

What does the study involve?
Participants will be divided into 3 cohorts:
• Participants who are eligible to receive AAV5-hRKp.RPGR in the second eye through surgery will be enrolled sequentially (one after the other) into 2 cohorts:
o Cohort 1: After receiving treatment, participants will be assessed for safety through Week 12.
o Cohort 2: Participants will receive the treatment once safety is determined in Cohort 1.
o Cohort 3: Participants who do not wish to undergo surgery or are not eligible for surgery.

Study consists of:
1. Screening/Baseline period (up to 6 months)
2. Administration of AAV5-hRKp.RPGR under the retina (Day 1): Participants may receive a dose

depending on the dosage administered in study MGT009 in the past.
3. Assessment period (52 weeks): Participants will undergo study assessments & tests including blood tests, imaging scans, eye examinations, efficacy outcome assessments & monitoring of side effects.
4. Long-term follow-up (4 years): The overall duration of the study will be approximately 5 years and 6 months.

What are the possible benefits and risks of participating?
There is no established benefit to participants of this study. Based on scientific theory, taking AAV5-hRKp.RPGR may improve eyesight. However, this cannot be guaranteed because AAV5-hRKp.RPGR is still under investigation as a treatment, and it is not known whether AAV5-hRKp.RPGR will work.

Participants may experience some benefit from participation in the study that is not due to receiving study drug, but due to regular visits and assessments monitoring overall health. Participation may help other people with X-linked Retinitis Pigmentosa in the future.

Participants may have side effects from the drugs or procedures used in this study that may be mild to severe and even life-threatening, and they can vary from person to person. Risks associated with standard surgical procedure itself, subretinal injection; those associated with gene therapy with a recombinant viral vector; adverse effects of corticosteroid prophylaxis (treatment given to prevent rejection of gene therapy) for immune suppression; and risks associated with study assessments. There are other, less frequent risks, like risks associated with the delivery of a recombinant viral vector. The participant information sheet and informed consent form, which will be signed by every participant agreeing to participate in the study, includes a detailed section outlining the known risks to participating in the study.

Not all possible side effects and risks related to AAV5-hRKp.RPGR are known at this moment. During the study, the sponsor may learn new information about AAV5-hRKp.RPGR. The study doctor will tell participants as soon as possible about any new information that might make them change their mind about being in the study, such as new risks.

To minimise the risk associated with taking part in the study, participants are frequently reviewed for any side effects and other medical events. Participants are educated to report any such events to their study doctor who will provide appropriate medical care. Any serious side effects that are reported to the sponsor are thoroughly reviewed by a specialist drug safety team.

There are no costs to participants to be in the study. The sponsor will pay for the study drug and tests that are part of the study. The participant will receive reasonable reimbursement for study related costs (e.g., travel/parking costs).

Where is the study run from?
MeiraGTx Ocular Limited (UK)

When is the study starting and how long is it expected to run for?
August 2024 to October 2030

Who is funding the study?
MeiraGTx Ocular Limited (UK)

Who is the main contact?
Lauren Ryan, lryan11@its.jnj.com
Prof. James Bainbridge, james.bainbridge1@nhs.net

Contact information

Type(s)
Principal investigator

Contact name
Prof James Bainbridge

Contact details
162 City Road
London
United Kingdom
EC1V 2PD
+44 207 566 2059
james.bainbridge1@nhs.net

Type(s)
Scientific, Public

Contact name
Mr Dominic Fitzsimmons

Contact details
MeiraGTx Ocular Limited, 34-38 Provost Street
London
United Kingdom
N1 7NH
+44 203 866 4320
dominic.fitzsimmons@meiragtx.com

Additional identifiers

ClinicalTrials.gov (NCT)
NCT06646289

Integrated Research Application System (IRAS)
1010255

Central Portfolio Management System (CPMS)
62454

Protocol serial number
74765340RPG2001

Study information

Scientific Title
An open-label, multicenter, phase 2 follow-on study for second eye treatment of patients previously treated with a recombinant Adeno-associated Virus Vector (AAV5 hRKp.RPGR) for gene therapy of adults and children with x-linked retinitis pigmentosa owing to defects in Retinitis Pigmentosa GTPase Regulator (RPGR)

Acronym
RPG2001

Study objectives
Primary objective:
To assess how safe and tolerable it is to deliver AAV5- hRKp.RPGR under the retina (back part of eye that senses light) into a single eye.

Secondary objectives:
To assess whether AAV5-hRKp.RPGR improves the vision-related activities and to assess the following changes after taking treatment with AAV5- hRKp.RPGR:
1. How well the retina works (retinal function)
2. How well a person can use eyesight in daily life (functional vision)
3. How well a person can see (visual function)

Ethics approval required
Ethics approval required

Ethics approval(s)
Approved 01/10/2024, West London & GTAC Research Ethics Committee (2 Redman Place, Stratford, London, E20 1JQ, United Kingdom; +44 2071048075; westlondon.rec@hra.nhs.uk), ref: 24/LO/0470

Primary study design
Interventional

Study design
Interventional non randomized

Study type(s)
Efficacy, Safety

Health condition(s) or problem(s) studied
X-Linked Retinitis Pigmentosa

Interventions
Experimental: Cohort 1 Participants will receive a dose of AAV5 hRKp.RPGR under the retina (low-dose or intermediate-dose) on Day 1 depending on the dosage administered in study MGT009 (NCT03252847) in the past. After receiving
the treatment, participants will be assessed for safety. Assigned interventions: Biological /Vaccine: AAV5-hRKp.RPGR. AAV5-hRKp.RPGR will be administered sub-retinally. Other Names:• JNJ-74765340, botaretigene sparoparvovec

Experimental: Cohort 2 Participants will receive the treatment dose of AAV5 hRKp.RPGR under the retina (low-dose or intermediate-dose) on Day 1 in the second eye once the safety will be determined in Cohort 1.
Assigned interventions: Biological/Vaccine: AAV5-hRKp.RPGR. AAV5-hRKp.RPGR will be administered sub-retinally. Other Names: JNJ-74765340, botaretigene sparoparvovec

Cohort 3 The participants who are not willing to undergo surgery or are not eligible for surgery will be assessed in this cohort. They will be assessed yearly until 5 years after their initial eye surgery in previous study MGT009.
No intervention (Follow-Up assessment). Participants will not receive any intervention and will undergo follow-up assessment.

Intervention Type
Drug

Phase
Phase II

Drug/device/biological/vaccine name(s)
AAV5-hRKp.RPGR

Primary outcome(s)
1. Number of Participants With Adverse Events (AE): From baseline up to 5.5 years

Key secondary outcome(s)
1. Change from Baseline in Mean Retinal Sensitivity Within the Central 10 Degrees (MRS10) to Months 12, 24, 36, 48 and 60
2. Change in Retinal Function as Assessed by Pointwise Response in Static Perimetry. From Month 12 up to Month 60
3. Change in Retinal Function as Assessed by Pointwise Response in Static Perimetry Within Central 30 Degrees Visual Field. From Month 12 up to Month 60
4. Change from Baseline in Mean Retinal Sensitivity Within the Full Visual Field (MRS90) to Months 12, 24, 36, 48 and 60
5. Change from Baseline in the Low Luminance Questionnaire (LLQ) Domain Scores to Month 12
6. Change from Baseline in Low Luminance Visual Acuity (LLVA) by Early Treatment Diabetic Retinopathy Study (ETDRS) Chart Letter Score to Months 12, 24, 36, 48 and 60
7. Change from Baseline in Best Corrected Visual Acuity (BCVA) by ETDRS Chart Letter Scores to Months 12, 24, 36, 48 and 60

Completion date
24/10/2030

Eligibility

Key inclusion criteria
1. Have been treated with AAV5-hRKp.RPGR in study MGT009 and have completed or is currently enrolled in Study MGT010-
2. Must sign an informed consent form indicating that they understand the purpose and procedures of the study and is willing to participate in the study
3. Willing to adhere to the protocol and long-term follow-up

Participant type(s)
Patient

Healthy volunteers allowed
No

Age group
Adult

Sex
Male

Total final enrolment
13

Key exclusion criteria
There are no specific exclusion criteria to enroll in this study

Date of first enrolment
25/04/2025

Date of final enrolment
31/10/2025

Locations

Countries of recruitment
United Kingdom

Study participating centre
NIHR Moorfields Biomedical Research Centre
Moorfields Eye Hospital NHS Foundation Trust
162 City Road
London
England
EC1V 2PD

Sponsor information

Organisation
MeiraGTx Ocular Limited

Funder(s)

Funder type
Industry

Funder Name
MeiraGTx Ocular Limited

Results and Publications

Individual participant data (IPD) sharing plan
The data sharing policy of Johnson & Johnson Innovative Medicine is available at innovativemedicine.jnj.com/clinical-trials/transparency. As noted on this site, requests for access to the study data can be submitted through Yale Open Data Access (YODA) Project site at yoda.yale.edu

IPD sharing plan summary
Available on request

Study outputs

Output type	Details	Date created	Date added	Peer reviewed?	Patient-facing?
HRA research summary			18/11/2026	No	No