Clinical trial results:
Long-term Follow-up Study of Participants Following an Open-label, Multi-center, Phase I/II Dose Escalation Trial of 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)

Summary

EudraCT number	2018-000425-31
Trial protocol	GB Outside EU/EEA
Global end of trial date	17 September 2025

Results information

Result version number	v1 (current)
This version publication date	29 March 2026
First version publication date	29 March 2026

Trial information

Trial identification

Sponsor protocol code	MGT010

Additional study identifiers

ISRCTN number	-
ClinicalTrials.gov id (NCT number)	NCT04312672
WHO universal trial number (UTN)	-

Notes:

Sponsors

Sponsor organisation name	Janssen Research & Development
Sponsor organisation address	Turnhoutseweg 30, Beerse, Belgium, B-2340
Public contact	Clinical Registry Group, Janssen Research & Development, ClinicalTrialsEU@its.jnj.com
Scientific contact	Clinical Registry Group, Janssen Research & Development, ClinicalTrialsEU@its.jnj.com

Notes:

Paediatric regulatory details

Is trial part of an agreed paediatric investigation plan (PIP)	Yes
EMA paediatric investigation plan number(s)	EMEA-002827-PIP01-20
Does article 45 of REGULATION (EC) No 1901/2006 apply to this trial?	No
Does article 46 of REGULATION (EC) No 1901/2006 apply to this trial?	Yes

Notes:

Results analysis stage

Analysis stage	Final
Date of interim/final analysis	23 November 2025
Is this the analysis of the primary completion data?	No

Global end of trial reached?	Yes
Global end of trial date	17 September 2025
Was the trial ended prematurely?	No

Notes:

General information about the trial

Main objective of the trial:

The main objective of the trial was to assess the longer term safety of AAV5-hRKp.RPGR administered to participants in the MGT009 trial, measured by the presence or absence of adverse events (AEs), the assessment of visual acuity, and loss of light perception.

Protection of trial subjects:

This study was conducted in accordance with the ethical principles that have their origin in the Declaration of Helsinki and that are consistent with Good Clinical Practice and applicable regulatory requirements.

Background therapy: -

Evidence for comparator: -

Actual start date of recruitment	19 February 2019
Long term follow-up planned	No
Independent data monitoring committee (IDMC) involvement?	No

Notes:

Population of trial subjects

Subjects enrolled per country

Country: Number of subjects enrolled	United Kingdom: 29
Country: Number of subjects enrolled	United States: 13
Worldwide total number of subjects	42
EEA total number of subjects	0

Notes:

Subjects enrolled per age group

In utero	0
Preterm newborn - gestational age < 37 wk	0
Newborns (0-27 days)	0
Infants and toddlers (28 days-23 months)	0
Children (2-11 years)	1
Adolescents (12-17 years)	2
Adults (18-64 years)	39
From 65 to 84 years	0
85 years and over	0

Subject disposition

Recruitment

Recruitment details: -

Pre-assignment

Screening details:

A total of 42 participants (including 3 children) were enrolled.

Period 1

Period 1 title	Overall study (overall period)
Is this the baseline period?	Yes
Allocation method	Not applicable
Blinding used	Not blinded

Arms

Are arms mutually exclusive?	No
Arm title	Low Dose AAV5-hRKp.RPGR

Arm description:

Participants from MGT009 study (from deferred and immediate treatment arm) were followed up for subretinal administration of a single monocular injection of up to 1 milliliter (mL) dose concentration 1.0*10^11 viral genomes per milliliter (vg/mL) of AAV5-hRKp.RPGR AAV5-hRKp.RPGR: AAV gene therapy for defects in the Retinitis Pigmentosa GTPase Regulator (RPGR) gene received during study

Arm type	Experimental
Investigational medicinal product name	AAV5-hRKp.RPGR
Investigational medicinal product code	
Other name	
Pharmaceutical forms	Injection
Routes of administration	Ocular use

Dosage and administration details:

No intervention was administered in the current study. Participants who received AAV5-hRKp.RPGR in MGT009 study were followed up in the current study.

Arm title	Intermediate Dose AAV5-hRKp.RPGR

Arm description:

Participants from MGT009 study (from deferred and immediate treatment arm) were followed up for subretinal administration of a single monocular injection of up to 1mL intermediate dose concentration 2.0*10^11 vg/mL of AAV5-hRKp.RPGR AAV5-hRKp.RPGR: AAV gene therapy for defects in the RPGR gene received during study MGT009.

Arm type	Experimental
Investigational medicinal product name	AAV5-hRKp.RPGR
Investigational medicinal product code	
Other name	
Pharmaceutical forms	Injection
Routes of administration	Ocular use

Dosage and administration details:

No intervention was administered in the current study. Participants who received AAV5-hRKp.RPGR in MGT009 study were followed up in the current study.

Arm title	High Dose AAV5-hRKp.RPGR

Arm description:

Participants from MGT009 study (from deferred and immediate treatment arm) were followed up for subretinal administration of a single monocular injection of up to 1mL high dose concentration 4.0*10^11 vg/mL of RPGR AAV5-hRKp.RPGR: AAV gene therapy for defects in the RPGR gene received during study MGT009.

Arm type	Experimental

Investigational medicinal product name	AAV5-hRKp.RPGR
Investigational medicinal product code	
Other name	
Pharmaceutical forms	Injection
Routes of administration	Ocular use

Dosage and administration details:

No intervention was administered in the current study. Participants who received AAV5-hRKp.RPGR in MGT009 study were followed up in the current study.

Number of subjects in period 1	Low Dose AAV5-hRKp.RPGR	Intermediate Dose AAV5-hRKp.RPGR	High Dose AAV5-hRKp.RPGR
Started	17	23	2
Completed	9	14	1
Not completed	8	9	1
Adverse event, serious fatal	-	1	-
Consent withdrawn by subject	1	-	1
Unspecified	7	8	-

Baseline characteristics

Reporting groups	
Reporting group title	Low Dose AAV5-hRKp.RPGR
Reporting group description:	
Participants from MGT009 study (from deferred and immediate treatment arm) were followed up for subretinal administration of a single monocular injection of up to 1 milliliter (mL) dose concentration 1.0*10^11 viral genomes per milliliter (vg/mL) of AAV5-hRKp.RPGR AAV5-hRKp.RPGR: AAV gene therapy for defects in the Retinitis Pigmentosa GTPase Regulator (RPGR) gene received during study	
Reporting group title	Intermediate Dose AAV5-hRKp.RPGR
Reporting group description:	
Participants from MGT009 study (from deferred and immediate treatment arm) were followed up for subretinal administration of a single monocular injection of up to 1mL intermediate dose concentration 2.0*10^11 vg/mL of AAV5-hRKp.RPGR AAV5-hRKp.RPGR: AAV gene therapy for defects in the RPGR gene received during study MGT009.	
Reporting group title	High Dose AAV5-hRKp.RPGR
Reporting group description:	
Participants from MGT009 study (from deferred and immediate treatment arm) were followed up for subretinal administration of a single monocular injection of up to 1mL high dose concentration 4.0*10^11 vg/mL of RPGR AAV5-hRKp.RPGR: AAV gene therapy for defects in the RPGR gene received during study MGT009.	

Reporting group values	Low Dose AAV5-hRKp.RPGR	Intermediate Dose AAV5-hRKp.RPGR	High Dose AAV5-hRKp.RPGR
Number of subjects	17	23	2
Age categorical			
Units: Subjects			
In Utero	0	0	0
Preterm newborn infants (gestional age < 37 wks)	0	0	0
Newborns (0-27 days)	0	0	0
Infants and toddlers (28 days - 23 months)	0	0	0
Children (2 - 11 years)	0	1	0
12 - 17 years	0	2	0
Adults (18 - 64 years)	17	20	2
From 65 - 84 years	0	0	0
85 years and over	0	0	0
Age continuous			
Units: years			
arithmetic mean	30.1	26.8	21.0
standard deviation	± 12.6	± 9.48	± 4.24
Gender categorical			
Units: Subjects			
Male	17	23	2
Female	0	0	0

Reporting group values	Total		
Number of subjects	42		
Age categorical			
Units: Subjects			
In Utero	0		

Preterm newborn infants (gestional age < 37 wks)	0		
Newborns (0-27 days)	0		
Infants and toddlers (28 days - 23 months)	0		
Children (2 - 11 years)	1		
12 - 17 years	2		
Adults (18 - 64 years)	39		
From 65 - 84 years	0		
85 years and over	0		
Age continuous			
Units: years			
arithmetic mean			
standard deviation	-		
Gender categorical			
Units: Subjects			
Male	42		
Female	0		

End points

End points reporting groups

Reporting group title	Low Dose AAV5-hRKp.RPGR

Reporting group description:

Participants from MGT009 study (from deferred and immediate treatment arm) were followed up for subretinal administration of a single monocular injection of up to 1 milliliter (mL) dose concentration 1.0*10^11 viral genomes per milliliter (vg/mL) of AAV5-hRKp.RPGR AAV5-hRKp.RPGR: AAV gene therapy for defects in the Retinitis Pigmentosa GTPase Regulator (RPGR) gene received during study

Reporting group title	Intermediate Dose AAV5-hRKp.RPGR

Reporting group description:

Participants from MGT009 study (from deferred and immediate treatment arm) were followed up for subretinal administration of a single monocular injection of up to 1mL intermediate dose concentration 2.0*10^11 vg/mL of AAV5-hRKp.RPGR AAV5-hRKp.RPGR: AAV gene therapy for defects in the RPGR gene received during study MGT009.

Reporting group title	High Dose AAV5-hRKp.RPGR

Reporting group description:

Participants from MGT009 study (from deferred and immediate treatment arm) were followed up for subretinal administration of a single monocular injection of up to 1mL high dose concentration 4.0*10^11 vg/mL of RPGR AAV5-hRKp.RPGR: AAV gene therapy for defects in the RPGR gene received during study MGT009.

Subject analysis set title	Low Dose AAV5-hRKp.RPGR
Subject analysis set type	Safety analysis

Subject analysis set description:

Participants from MGT009 study (from deferred and immediate treatment arm) were followed up for subretinal administration of a single monocular injection of up to 1 milliliter (mL) dose concentration 1.0*10^11 viral genomes per milliliter (vg/mL) of AAV5-hRKp.RPGR AAV5-hRKp.RPGR: AAV gene therapy for defects in the Retinitis Pigmentosa GTPase Regulator (RPGR) gene received during study

Subject analysis set title	Intermediate Dose AAV5-hRKp.RPGR
Subject analysis set type	Safety analysis

Subject analysis set description:

Participants from MGT009 study (from deferred and immediate treatment arm) were followed up for subretinal administration of a single monocular injection of up to 1mL intermediate dose concentration 2.0*10^11 vg/mL of AAV5-hRKp.RPGR AAV5-hRKp.RPGR: AAV gene therapy for defects in the RPGR gene received during study MGT009.

Subject analysis set title	High Dose AAV5-hRKp.RPGR
Subject analysis set type	Safety analysis

Subject analysis set description:

Participants from MGT009 study (from deferred and immediate treatment arm) were followed up for subretinal administration of a single monocular injection of up to 1mL high dose concentration 4.0*10^11 vg/mL of RPGR AAV5-hRKp.RPGR: AAV gene therapy for defects in the RPGR gene received during study MGT009.

Primary: Number of Participants With Treatment-emergent Adverse Events (TEAEs)

End point title	Number of Participants With Treatment-emergent Adverse Events (TEAEs)[1]

End point description:

An adverse event (AE) was any untoward medical occurrence in a clinical study participant administered a medicinal (investigational or non-investigational) product. AE does not necessarily have a causal relationship with intervention. Any AE occurring at or after the initial administration of AAV5-hRKp.RPGR was considered to be treatment-emergent. TEAEs included both serious and non-serious adverse events. Safety analysis set (SAS) included all enrolled participants who were administered bota-vec in Study MGT009 and consented to Study MGT010, and performed separately for each dose level and overall, unless otherwise specified.

End point type	Primary

End point timeframe:

For Deferred treatment arm: From Month 6 post treatment in study MGT009 up to 60 months; For Immediate treatment arm: From Month 12 post treatment in study MGT009 up to 60 months

Notes:

[1] - No statistical analyses have been specified for this primary end point. It is expected there is at least one statistical analysis for each primary end point.
Justification: Only descriptive data was planned to be reported for this endpoint.

End point values	Low Dose AAV5-hRKp.RPGR	Intermediate Dose AAV5-hRKp.RPGR	High Dose AAV5-hRKp.RPGR	
Subject group type	Subject analysis set	Subject analysis set	Subject analysis set	
Number of subjects analysed	17	22	3	
Units: Subjects	17	20	3	

Statistical analyses

No statistical analyses for this end point

Secondary: Change From Baseline in Best Corrected Visual Acuity (BCVA) Using the Early Treatment Diabetic Retinopathy Study (ETDRS) Chart Letter Score

End point title	Change From Baseline in Best Corrected Visual Acuity (BCVA) Using the Early Treatment Diabetic Retinopathy Study (ETDRS) Chart Letter Score

End point description:

Change from baseline in BCVA by ETDRS chart letter score in monocular assessment was reported. BCVA was determined using ETDRS chart by counting number of ETDRS letters read under normal lighting conditions. ETDRS chart letter score ranged from 0 to 100 letters. 20/20 snellen was equivalent to 85 letters on the ETDRS chart or 0 logarithm of the minimum angle of resolution (logMAR). Higher ETDRS score indicated better vision. Full analysis set (FAS) included all enrolled adults and pediatric participants treated with AAV5-hRKp. RPGR and completed both at least one baseline visit prior to treatment in study MGT009 and at least one visit in study MGT010. 'n' (number analyzed) signifies number of participants analyzed at specified timepoints. 99999 signifies that standard deviation was not estimable for single participant.

End point type	Secondary

End point timeframe:

Baseline, Month 12, Month 18, Month 24, Month 36, Month 48, and Month 60

End point values	Low Dose AAV5-hRKp.RPGR	Intermediate Dose AAV5-hRKp.RPGR	High Dose AAV5-hRKp.RPGR	
Subject group type	Reporting group	Reporting group	Reporting group	
Number of subjects analysed	17	23	2	
Units: Number of ETDRS letters				
arithmetic mean (standard deviation)				
At Month 12 (n=15,22,2)	1.4 (± 4.02)	0.2 (± 5.68)	-5.0 (± 0.47)	
At Month 18 (n=15,23,2)	-1.8 (± 7.51)	0.0 (± 5.09)	-6.5 (± 1.65)	
At Month 24 (n=16,21,2)	-2.6 (± 5.51)	-1.7 (± 7.97)	-7.5 (± 0.24)	
At Month 36 (n=16,23,2)	-4.8 (± 9.40)	-5.6 (± 10.10)	-8.0 (± 0.47)	
At Month 48 (n=15,22,2)	-4.3 (± 8.41)	-6.4 (± 13.12)	-16.5 (± 5.42)	
At Month 60 (n=9,13,1)	-8.6 (± 13.58)	-13.0 (± 17.74)	-14.7 (± 99999)	

Statistical analyses

No statistical analyses for this end point

Secondary: Change From Baseline in Low Luminance Visual Acuity (LLVA) as Assessed by ETDRS Chart Letter Score

End point title	Change From Baseline in Low Luminance Visual Acuity (LLVA) as Assessed by ETDRS Chart Letter Score

End point description:

Change from baseline in LLVA by ETDRS chart letter score in monocular assessment were reported. LLVA was determined using ETDRS chart by counting the number of ETDRS letters read under low light conditions. ETDRS chart letter score ranged from 0 to 100 letters. 20/20 snellen was equivalent to 85 letters on ETDRS chart or 0 logMAR. Higher ETDRS score indicated better vision. FAS included all enrolled adults and pediatric participants treated with AAV5-hRKp.RPGR and completed both at least one baseline visit prior to treatment in study MGT009 and at least one visit in study MGT010. 'N' (Overall number of participants analyzed) signifies number of participants evaluable for this endpoint. 'n' (number analyzed) signifies number of participants analyzed at specified timepoints.

End point type	Secondary

End point timeframe:

Baseline, Month 12, Month 18, Month 24, Month 36, Month 48, and Month 60

End point values	Low Dose AAV5-hRKp.RPGR	Intermediate Dose AAV5-hRKp.RPGR	High Dose AAV5-hRKp.RPGR	
Subject group type	Reporting group	Reporting group	Reporting group	
Number of subjects analysed	9	9	0[2]	
Units: Number of ETDRS letters				
arithmetic mean (standard deviation)				
At Month 12 (n=8,8,0)	2.9 (± 4.39)	-1.0 (± 15.57)	()	
At Month 18 (n=8,6,0)	-2.4 (± 8.12)	1.8 (± 8.90)	()	
At Month 24 (n=9,8,0)	-3.2 (± 8.91)	-4.3 (± 13.74)	()	
At Month 36 (n=9,9,0)	-4.7 (± 14.70)	-12.7 (± 16.66)	()	
At Month 48 (n=7,8,0)	-5.5 (± 16.04)	-8.5 (± 16.65)	()	
At Month 60 (n=3,3,0)	-17.6 (± 30.86)	-31.1 (± 19.17)	()	

Notes:

[2] - LLVA data not analyzable for any subject in the highdose group; thus, no subjects were analyzed.

Statistical analyses

No statistical analyses for this end point

Secondary: Change From Baseline in Mean Retinal Sensitivity Within the Central 10 Degree Visual Field Excluding Scotoma (MRS10) in Static Perimetry

End point title	Change From Baseline in Mean Retinal Sensitivity Within the

	Central 10 Degree Visual Field Excluding Scotoma (MRS10) in Static Perimetry

End point description:

Change from baseline in mean retinal sensitivity within the central 10 degrees excluding scotoma (MRS10) in static perimetry was reported. FAS included all enrolled adults and pediatric participants treated with AAV5-hRKp.RPGR and completed both at least one baseline visit prior to treatment in study MGT009 and at least one visit in study MGT010. Here 'n' (number analyzed) signifies number of participants analyzed at specified timepoints. In 'High Dose AAV5-hRKp.RPGR' arm, at month 60, 99999 signifies that standard deviation was not estimable for single participant.

End point type	Secondary

End point timeframe:

Baseline, Month 12, Month 18, Month 24, Month 36, Month 48, and Month 60

End point values	Low Dose AAV5-hRKp.RPGR	Intermediate Dose AAV5-hRKp.RPGR	High Dose AAV5-hRKp.RPGR	
Subject group type	Reporting group	Reporting group	Reporting group	
Number of subjects analysed	17	23	2	
Units: Decibels (dB)				
arithmetic mean (standard deviation)				
At Month 12 (n=15,20,2)	1.0 (± 2.09)	1.5 (± 2.62)	-0.9 (± 3.15)	
At Month 18 (n=15,20,2)	0.1 (± 2.43)	0.4 (± 3.07)	-3.8 (± 4.41)	
At Month 24 (n=16,20,2)	-1.0 (± 3.12)	0.4 (± 2.84)	-2.6 (± 5.41)	
At Month 36 (n=17,23,2)	-1.7 (± 3.97)	-0.8 (± 3.54)	-4.2 (± 4.23)	
At Month 48 (n=15,20,2)	-2.7 (± 4.60)	-1.8 (± 3.03)	-6.6 (± 4.68)	
At Month 60 (n=9,13,1)	-3.0 (± 4.24)	-3.0 (± 3.03)	-11.9 (± 99999)	

Statistical analyses

No statistical analyses for this end point

Secondary: Percentage of Responders in Pointwise Data in Static Perimetry Within the Full Visual Field

End point title	Percentage of Responders in Pointwise Data in Static Perimetry Within the Full Visual Field

End point description:

Percentage of responders in pointwise data in static perimetry within the full visual field was reported. A responder was defined as a participant with greater than equal to (>=) 7dB improvement from baseline in at least 5 loci at the current visit and another visit prior. FAS included all enrolled adults and pediatric participants treated with AAV5-hRKp.RPGR and completed both at least one baseline visit prior to treatment in study MGT009 and at least one visit in study MGT010. Here 'n' (number analyzed) signifies number of participants analyzed at specified timepoints.

End point type	Secondary

End point timeframe:

At Month 12, Month 18, Month 24, Month 36, Month 48, and Month 60

End point values	Low Dose AAV5-hRKp.RPGR	Intermediate Dose AAV5-hRKp.RPGR	High Dose AAV5-hRKp.RPGR	
Subject group type	Reporting group	Reporting group	Reporting group	
Number of subjects analysed	17	23	2	
Units: Percentage of responders				
number (not applicable)				
At Month 12 (n=15,20,2)	60.0	65.0	50.0	
At Month 18 (n=15,20,2)	46.7	75.0	0	
At Month 24 (n=16,20,2)	50.0	70.0	50.0	
At Month 36 (n=17,23,2)	47.1	52.2	50.0	
At Month 48 (n=15,20,2)	40.0	35.0	0	
At Month 60 (n=9,13,1)	22.2	0	0	

Statistical analyses

No statistical analyses for this end point

Secondary: Percentage of Responder in Pointwise Data in Static Perimetry Within the Central 30-Degree Visual Field

End point title	Percentage of Responder in Pointwise Data in Static Perimetry Within the Central 30-Degree Visual Field

End point description:

Percentage of responder in pointwise data in static perimetry within the central 30-degree visual field was reported. FAS included all enrolled adults and pediatric participants treated with AAV5-hRKp.RPGR and completed both at least one baseline visit prior to treatment in study MGT009 and at least one visit in study MGT010. Here 'n' (number analyzed) signifies number of participants analyzed at specified timepoints.

End point type	Secondary

End point timeframe:

At Month 12, Month 18, Month 24, Month 36, Month 48, and Month 60

End point values	Low Dose AAV5-hRKp.RPGR	Intermediate Dose AAV5-hRKp.RPGR	High Dose AAV5-hRKp.RPGR	
Subject group type	Reporting group	Reporting group	Reporting group	
Number of subjects analysed	17	23	2	
Units: Percentage of responders				
number (not applicable)				
At Month 12 (n=15,20,2)	46.7	45.0	0	
At Month 18 (n=15,20,2)	26.7	45.0	0	
At Month 24 (n=16,20,2)	37.5	50.0	50.0	
At Month 36 (n=17,23,2)	29.4	30.4	50.0	
At Month 48 (n=15,20,2)	26.7	25.0	0	
At Month 60 (n=9, 13, 1)	11.1	0	0	

Statistical analyses

No statistical analyses for this end point

Adverse events

Adverse events information

Timeframe for reporting adverse events:

For Deferred treatment arm: From Month 6 post treatment in study MGT009 up to 60 months; For Immediate treatment arm: From Month 12 post treatment in study MGT009 up to 60 months.

Adverse event reporting additional description:

Safety analysis set (SAS) included all enrolled participants who administered bota-vec in Study MGT009 and consented to Study MGT010, and performed separately for each dose level and overall, unless otherwise specified.

Assessment type	Non-systematic

Dictionary used

Dictionary name	MedDRA
Dictionary version	28.1

Reporting groups

Reporting group title	Low Dose AAV5-hRKp.RPGR

Reporting group description:

Participants from MGT009 study (from deferred and immediate treatment arm) were followed up for subretinal administration of a single monocular injection of up to 1 milliliter (mL) dose concentration 1.0*10^11 viral genomes per milliliter (vg/mL) of AAV5-hRKp.RPGR AAV5-hRKp.RPGR: AAV gene therapy for defects in the Retinitis Pigmentosa GTPase Regulator (RPGR) gene received during study

Reporting group title	Intermediate Dose AAV5-hRKp.RPGR

Reporting group description:

Participants from MGT009 study (from deferred and immediate treatment arm) were followed up for subretinal administration of a single monocular injection of up to 1mL intermediate dose concentration 2.0*10^11 vg/mL of AAV5-hRKp.RPGR AAV5-hRKp.RPGR: AAV gene therapy for defects in the RPGR gene received during study MGT009.

Reporting group title	High Dose AAV5-hRKp.RPGR

Reporting group description:

Participants from MGT009 study (from deferred and immediate treatment arm) were followed up for subretinal administration of a single monocular injection of up to 1mL high dose concentration 4.0*10^11 vg/mL of RPGR AAV5-hRKp.RPGR: AAV gene therapy for defects in the RPGR gene received during study MGT009.

Serious adverse events	Low Dose AAV5-hRKp.RPGR	Intermediate Dose AAV5-hRKp.RPGR	High Dose AAV5-hRKp.RPGR
Total subjects affected by serious adverse events			
subjects affected / exposed	1 / 17 (5.88%)	2 / 22 (9.09%)	0 / 3 (0.00%)
number of deaths (all causes)	0	1	0
number of deaths resulting from adverse events	0	1	0
Eye disorders			
Noninfective Chorioretinitis			
subjects affected / exposed	0 / 17 (0.00%)	1 / 22 (4.55%)	0 / 3 (0.00%)
occurrences causally related to treatment / all	0 / 0	0 / 1	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Uveitis			

subjects affected / exposed	1 / 17 (5.88%)	0 / 22 (0.00%)	0 / 3 (0.00%)
occurrences causally related to treatment / all	0 / 1	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Psychiatric disorders			
Completed Suicide			
subjects affected / exposed	0 / 17 (0.00%)	1 / 22 (4.55%)	0 / 3 (0.00%)
occurrences causally related to treatment / all	0 / 0	0 / 1	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 1	0 / 0

Frequency threshold for reporting non-serious adverse events: 5 %

Non-serious adverse events	Low Dose AAV5-hRKp.RPGR	Intermediate Dose AAV5-hRKp.RPGR	High Dose AAV5-hRKp.RPGR
Total subjects affected by non-serious adverse events			
subjects affected / exposed	17 / 17 (100.00%)	20 / 22 (90.91%)	3 / 3 (100.00%)
Surgical and medical procedures			
Intraocular Lens Implant			
subjects affected / exposed	1 / 17 (5.88%)	1 / 22 (4.55%)	0 / 3 (0.00%)
occurrences (all)	2	2	0
Cataract Operation			
subjects affected / exposed	1 / 17 (5.88%)	1 / 22 (4.55%)	0 / 3 (0.00%)
occurrences (all)	2	2	0
General disorders and administration site conditions			
Pyrexia			
subjects affected / exposed	0 / 17 (0.00%)	2 / 22 (9.09%)	0 / 3 (0.00%)
occurrences (all)	0	2	0
Peripheral Swelling			
subjects affected / exposed	1 / 17 (5.88%)	0 / 22 (0.00%)	0 / 3 (0.00%)
occurrences (all)	1	0	0
Immune system disorders			
Seasonal Allergy			
subjects affected / exposed	0 / 17 (0.00%)	2 / 22 (9.09%)	0 / 3 (0.00%)
occurrences (all)	0	2	0
Psychiatric disorders			
Attention Deficit Hyperactivity Disorder			

subjects affected / exposed	1 / 17 (5.88%)	2 / 22 (9.09%)	0 / 3 (0.00%)
occurrences (all)	1	2	0
Depression			
subjects affected / exposed	1 / 17 (5.88%)	0 / 22 (0.00%)	0 / 3 (0.00%)
occurrences (all)	1	0	0
Mania			
subjects affected / exposed	1 / 17 (5.88%)	0 / 22 (0.00%)	0 / 3 (0.00%)
occurrences (all)	1	0	0
Mood Altered			
subjects affected / exposed	1 / 17 (5.88%)	0 / 22 (0.00%)	0 / 3 (0.00%)
occurrences (all)	1	0	0
Investigations			
Alanine Aminotransferase Increased			
subjects affected / exposed	1 / 17 (5.88%)	1 / 22 (4.55%)	0 / 3 (0.00%)
occurrences (all)	1	1	0
Low Density Lipoprotein Increased			
subjects affected / exposed	1 / 17 (5.88%)	0 / 22 (0.00%)	0 / 3 (0.00%)
occurrences (all)	1	0	0
Intraocular Pressure Increased			
subjects affected / exposed	1 / 17 (5.88%)	0 / 22 (0.00%)	0 / 3 (0.00%)
occurrences (all)	3	0	0
Fundus Autofluorescence			
subjects affected / exposed	0 / 17 (0.00%)	0 / 22 (0.00%)	1 / 3 (33.33%)
occurrences (all)	0	0	1
Aspartate Aminotransferase Increased			
subjects affected / exposed	1 / 17 (5.88%)	0 / 22 (0.00%)	0 / 3 (0.00%)
occurrences (all)	1	0	0
Injury, poisoning and procedural complications			
Skin Laceration			
subjects affected / exposed	2 / 17 (11.76%)	0 / 22 (0.00%)	0 / 3 (0.00%)
occurrences (all)	2	0	0
Joint Dislocation			
subjects affected / exposed	1 / 17 (5.88%)	0 / 22 (0.00%)	0 / 3 (0.00%)
occurrences (all)	1	0	0
Cardiac disorders			

Atrial Fibrillation			
subjects affected / exposed	1 / 17 (5.88%)	0 / 22 (0.00%)	0 / 3 (0.00%)
occurrences (all)	1	0	0
Nervous system disorders			
Dizziness			
subjects affected / exposed	2 / 17 (11.76%)	0 / 22 (0.00%)	0 / 3 (0.00%)
occurrences (all)	2	0	0
Eye disorders			
Cataract Cortical			
subjects affected / exposed	1 / 17 (5.88%)	2 / 22 (9.09%)	0 / 3 (0.00%)
occurrences (all)	1	2	0
Cataract Subcapsular			
subjects affected / exposed	3 / 17 (17.65%)	5 / 22 (22.73%)	0 / 3 (0.00%)
occurrences (all)	6	11	0
Eye Pain			
subjects affected / exposed	2 / 17 (11.76%)	1 / 22 (4.55%)	0 / 3 (0.00%)
occurrences (all)	2	1	0
Epiretinal Membrane			
subjects affected / exposed	1 / 17 (5.88%)	2 / 22 (9.09%)	0 / 3 (0.00%)
occurrences (all)	1	2	0
Cataract			
subjects affected / exposed	3 / 17 (17.65%)	3 / 22 (13.64%)	1 / 3 (33.33%)
occurrences (all)	3	3	1
Aniseikonia			
subjects affected / exposed	1 / 17 (5.88%)	0 / 22 (0.00%)	0 / 3 (0.00%)
occurrences (all)	1	0	0
Anterior Chamber Cell			
subjects affected / exposed	2 / 17 (11.76%)	4 / 22 (18.18%)	0 / 3 (0.00%)
occurrences (all)	3	10	0
Cystoid Macular Oedema			
subjects affected / exposed	2 / 17 (11.76%)	2 / 22 (9.09%)	0 / 3 (0.00%)
occurrences (all)	2	2	0
Retinal Deposits			
subjects affected / exposed	2 / 17 (11.76%)	0 / 22 (0.00%)	0 / 3 (0.00%)
occurrences (all)	2	0	0
Retinal Haemorrhage			

subjects affected / exposed	1 / 17 (5.88%)	0 / 22 (0.00%)	0 / 3 (0.00%)
occurrences (all)	1	0	0
Retinal Degeneration			
subjects affected / exposed	0 / 17 (0.00%)	2 / 22 (9.09%)	0 / 3 (0.00%)
occurrences (all)	0	3	0
Posterior Capsule Opacification			
subjects affected / exposed	1 / 17 (5.88%)	3 / 22 (13.64%)	1 / 3 (33.33%)
occurrences (all)	2	3	1
Photophobia			
subjects affected / exposed	2 / 17 (11.76%)	1 / 22 (4.55%)	0 / 3 (0.00%)
occurrences (all)	2	1	0
Optic Atrophy			
subjects affected / exposed	1 / 17 (5.88%)	0 / 22 (0.00%)	0 / 3 (0.00%)
occurrences (all)	2	0	0
Metamorphopsia			
subjects affected / exposed	2 / 17 (11.76%)	3 / 22 (13.64%)	0 / 3 (0.00%)
occurrences (all)	2	4	0
Macular Pigmentary Changes			
subjects affected / exposed	1 / 17 (5.88%)	0 / 22 (0.00%)	0 / 3 (0.00%)
occurrences (all)	1	0	0
Macular Cyst			
subjects affected / exposed	1 / 17 (5.88%)	0 / 22 (0.00%)	0 / 3 (0.00%)
occurrences (all)	1	0	0
Uveitis			
subjects affected / exposed	0 / 17 (0.00%)	1 / 22 (4.55%)	1 / 3 (33.33%)
occurrences (all)	0	1	1
Iridocyclitis			
subjects affected / exposed	2 / 17 (11.76%)	0 / 22 (0.00%)	0 / 3 (0.00%)
occurrences (all)	2	0	0
Low Luminance Best-Corrected Visual Acuity Decreased			
subjects affected / exposed	0 / 17 (0.00%)	0 / 22 (0.00%)	1 / 3 (33.33%)
occurrences (all)	0	0	1
Vitreous Haemorrhage			
subjects affected / exposed	1 / 17 (5.88%)	0 / 22 (0.00%)	0 / 3 (0.00%)
occurrences (all)	1	0	0

Vitreous Floaters			
subjects affected / exposed	1 / 17 (5.88%)	0 / 22 (0.00%)	0 / 3 (0.00%)
occurrences (all)	1	0	0
Vitreal Cells			
subjects affected / exposed	0 / 17 (0.00%)	3 / 22 (13.64%)	1 / 3 (33.33%)
occurrences (all)	0	5	1
Visual Impairment			
subjects affected / exposed	1 / 17 (5.88%)	1 / 22 (4.55%)	0 / 3 (0.00%)
occurrences (all)	1	3	0
Visual Field Defect			
subjects affected / exposed	0 / 17 (0.00%)	0 / 22 (0.00%)	1 / 3 (33.33%)
occurrences (all)	0	0	1
Visual Acuity Reduced			
subjects affected / exposed	4 / 17 (23.53%)	9 / 22 (40.91%)	1 / 3 (33.33%)
occurrences (all)	6	18	1
Gastrointestinal disorders			
Abdominal Pain			
subjects affected / exposed	1 / 17 (5.88%)	0 / 22 (0.00%)	0 / 3 (0.00%)
occurrences (all)	1	0	0
Vomiting			
subjects affected / exposed	1 / 17 (5.88%)	0 / 22 (0.00%)	0 / 3 (0.00%)
occurrences (all)	1	0	0
Abdominal Pain Upper			
subjects affected / exposed	1 / 17 (5.88%)	1 / 22 (4.55%)	0 / 3 (0.00%)
occurrences (all)	1	1	0
Skin and subcutaneous tissue disorders			
Dermatitis Acneiform			
subjects affected / exposed	0 / 17 (0.00%)	0 / 22 (0.00%)	1 / 3 (33.33%)
occurrences (all)	0	0	1
Dry Skin			
subjects affected / exposed	0 / 17 (0.00%)	1 / 22 (4.55%)	1 / 3 (33.33%)
occurrences (all)	0	1	1
Pain of Skin			
subjects affected / exposed	1 / 17 (5.88%)	0 / 22 (0.00%)	0 / 3 (0.00%)
occurrences (all)	1	0	0
Rash			

subjects affected / exposed	1 / 17 (5.88%)	0 / 22 (0.00%)	0 / 3 (0.00%)
occurrences (all)	1	0	0
Musculoskeletal and connective tissue disorders			
Bone Loss			
subjects affected / exposed	0 / 17 (0.00%)	0 / 22 (0.00%)	1 / 3 (33.33%)
occurrences (all)	0	0	1
Musculoskeletal Stiffness			
subjects affected / exposed	0 / 17 (0.00%)	0 / 22 (0.00%)	1 / 3 (33.33%)
occurrences (all)	0	0	1
Osteopenia			
subjects affected / exposed	1 / 17 (5.88%)	0 / 22 (0.00%)	0 / 3 (0.00%)
occurrences (all)	1	0	0
Pain in Extremity			
subjects affected / exposed	1 / 17 (5.88%)	0 / 22 (0.00%)	0 / 3 (0.00%)
occurrences (all)	1	0	0
Ankylosing Spondylitis			
subjects affected / exposed	1 / 17 (5.88%)	0 / 22 (0.00%)	0 / 3 (0.00%)
occurrences (all)	1	0	0
Infections and infestations			
Chorioretinitis			
subjects affected / exposed	0 / 17 (0.00%)	2 / 22 (9.09%)	0 / 3 (0.00%)
occurrences (all)	0	2	0
Upper Respiratory Tract Infection			
subjects affected / exposed	1 / 17 (5.88%)	1 / 22 (4.55%)	0 / 3 (0.00%)
occurrences (all)	2	1	0
Sinusitis			
subjects affected / exposed	1 / 17 (5.88%)	0 / 22 (0.00%)	0 / 3 (0.00%)
occurrences (all)	1	0	0
Rhinitis			
subjects affected / exposed	1 / 17 (5.88%)	0 / 22 (0.00%)	0 / 3 (0.00%)
occurrences (all)	2	0	0
Lower Respiratory Tract Infection			
subjects affected / exposed	1 / 17 (5.88%)	1 / 22 (4.55%)	0 / 3 (0.00%)
occurrences (all)	1	3	0
Influenza			

subjects affected / exposed	1 / 17 (5.88%)	0 / 22 (0.00%)	0 / 3 (0.00%)
occurrences (all)	1	0	0
Ear Infection			
subjects affected / exposed	0 / 17 (0.00%)	2 / 22 (9.09%)	0 / 3 (0.00%)
occurrences (all)	0	2	0
Covid-19			
subjects affected / exposed	3 / 17 (17.65%)	5 / 22 (22.73%)	0 / 3 (0.00%)
occurrences (all)	3	5	0
Conjunctivitis			
subjects affected / exposed	1 / 17 (5.88%)	0 / 22 (0.00%)	0 / 3 (0.00%)
occurrences (all)	1	0	0
Metabolism and nutrition disorders			
Gout			
subjects affected / exposed	1 / 17 (5.88%)	0 / 22 (0.00%)	0 / 3 (0.00%)
occurrences (all)	1	0	0

More information

Substantial protocol amendments (globally)

Were there any global substantial amendments to the protocol? Yes

Date	Amendment
06 June 2019	The overall reason for this amendment was to update participant numbers and include a 12 visit to align with the updated visit schedule on the MGT009 study. Full field stimulus testing assessment was removed.
13 August 2020	The overall reason for this amendment was to include assessments that have been recently included in the RPGR gene therapy trial. Provide clarification around optional assessments.
15 September 2020	The overall reason for this amendment was correction of page numbering.
16 October 2020	The overall reason for this amendment was changing of vector name. Removal of information relating to caregiver patient reported outcomes (PROs) as not applicable to study. More detail on PRO information to align with treatment study protocol (MGT009).
09 June 2021	The overall reason for this amendment was to update the number of research sites. XLRP RPGR MGT009 trial design was updated to include randomisation phase. Remove Professor Bainbridge from the declaration of Interests section as he no longer holds share in MeiraGTx or receives payment for consultancy work.
07 April 2022	The overall reason for this amendment was to include language to allow alternative reduced follow-up options. Updated the number of participants expected to be included in this study (based on MGT009 data). Removed collection of medical history from the schedule of assessments. Indicated that all medical events that occurred between the last study visit in Study MGT009 and prior to signing informed consent for Study MGT010 are to be reported as AEs in Study MGT010. Replaced “EMAS” by “Sponsor or delegate”
03 April 2023	The overall reason for this amendment was to clarify that electroretinography testing will be discontinued after M18 if responses remain undetectable or if confirmed by the electroretinography reading centre as unnecessary. Added that an interim analysis of the data is planned and others may be undertaken. Clarified that the IDMC also considers data of other studies in the RPGR program. Added that unscheduled visits may be conducted to follow-up adverse events. Reworded text on optional assessments. Updated information related to the data management vendor. Clarified that the Sponsor will disclose the results of the clinical study as required by applicable regulations.
18 December 2023	The overall rationale for the amendment was to update sections of the protocol with additional or revised specifics such as changes to the secondary endpoints, addition of exploratory endpoints and to enable clarity and implement operational changes including sponsorship transfer from MeiraGTx UK II Ltd to Janssen Research & Development (JRD) and administrative changes (For example, updates to Janssen Research & Development protocol template format).

Notes:

Interruptions (globally)

Were there any global interruptions to the trial? No

Limitations and caveats

Limitations of the trial such as small numbers of subjects analysed or technical problems leading to unreliable data.

Study was not powered to evaluate efficacy, as all participants received bota-vec in previous study and there was no concurrent control arm in this study. Due to revision in scope, VMA, LLQ, and MRS90 endpoints were not analyzed and reported.

Notes: