GenSight BIOLOGICS

Corporate Presentation

March 2020

A LEADING Gene Therapy BIOTECHNOLOGY COMPANY

GENSIGHT-BIOLOGICS.COM

Disclaimer

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Corporate Overview – Transitioning from R&D to Commercial organization

GenSight at the forefront of Gene Therapy in ophthalmology

- Publicly traded Biotech company
- Seasoned management team with strong BioPharma and Financial markets experience
- Technology platform leveraging disruptive gene therapies in ophthalmology
- Lead product targets mitochondrial diseases, applicable to broad number of diseases
- Second compound targets large indications such as Dry Age-related Macular Degeneration
- ATU granted in France in December 2019

Differentiated Gene Therapy approach

- Focus on ophthalmological diseases:
  - Through mitochondrial DNA correction (LUMEVOQ™)
  - By shaping retinal ganglion cells into photoreceptors (GS030)
- Using AAV2 as vector with proven safety and efficiency in human as well as well established manufacturing process

LUMEVOQ™ (GS010) - Preparing for regulatory submission in 2020 and commercial launch in 2021

- Strong clinical benefit vs Natural History and/or nadir in 2 completed Phase III studies in rare ophthalmic disease with no/limited competition
- Commercial strategy and contract manufacturing capabilities close to completion

Established in 2012 / IPO in 2016	
EuroNext Paris:	SIGHT
Market Cap (Feb 28, 2020):	€ 77m
Avg 30-day Daily volume:	1.8% of O/S
Cash (Dec 31, 2019):	€ 19.2m

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Investment Case

Targeting the LHON ND4 market with high unmet medical need and no widely approved treatment

- Disease affects ~15,000/22,000 patients in the US/EU with 800/1,200 new cases each year
- Commercial strategy and manufacturing capabilities close to completion
- Bilateral injection priced at €700,000 / patient in French named patient Temporary Authorization for Use

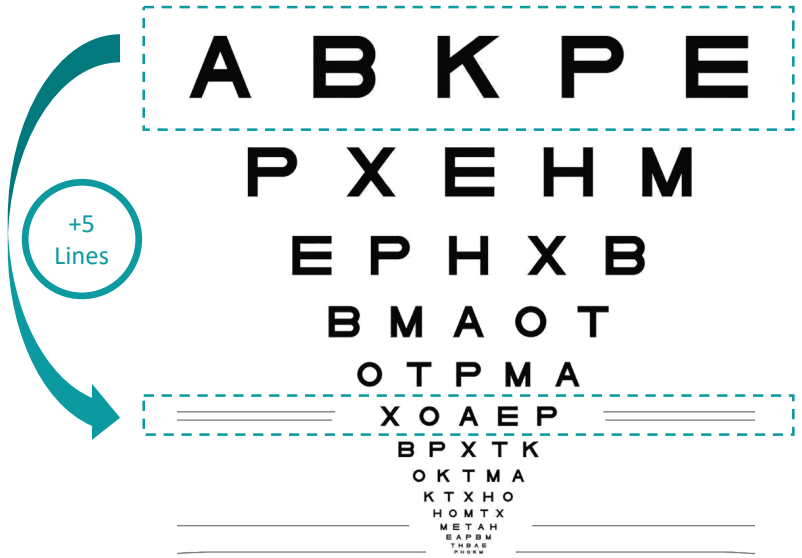
Unparalleled clinical benefit demonstrated with LUVEMOQ™ in Leber Hereditary Optic Neuropathy (LHON) in two Phase III studies

- +28/+26 ETDRS letters (i.e. over **5 lines** on visual scale) improvement vs nadir(1) in the two-Phase III studies
- Clinically meaningful improvement on all Quality of Life parameters at week 96

Additional opportunities through technology platform

- Large number of mitochondrial diseases making Mitochondrial Targeting Sequence (MTS used in GS010) a pipeline in itself
- GS030 in Retinitis pigmentosa and dry-AMD (Phase I/II)

Improvement vs nadir in REVERSE and RESCUE



(1) Nadir: worst visual acuity from baseline

Seasoned Executive Team

Bernard Gilly
Chief Executive Officer

PIXIUM VISION (Since 2011)
Chairman of the Board, Founder

FOVEA PHARMA (2005-2009)
Chairman & CEO – sold to Sanofi

SOFINNOVA PARTNERS (2000-2005)
Managing Partner

TRANSGENE (1992-2000)
Chairman & CEO

Ph.D. in biology and bio-economics

Thomas Gidoin
Chief Financial Officer

DBV TECHNOLOGIES (2012-2015)
VP of Finance

IPSEN (2008-2011)
UK Operations Controller (London)
Senior Financial Analyst (Paris)

ERNST & YOUNG (2007-2008)
Auditor

Magali Taiel
Chief Medical Officer

ProQR THERAPEUTICS (2016-2018)
VP of Clinical Development

ELI LILLY (2004-2016)
Medical Department Lead

PFIZER (2001-2004)
Medical Advisor

SERVIER (1999-2001)
R&D International Project Manager

MD, Board-certified ophthalmologist

Catherine Cancian
Vice President Pharmaceutical Operations

GENETHON (2015-2017)
Project Leader

SANOFI PASTEUR (1998-2014)
Industrial Operations and Regulatory Affairs

Julio Benedicto
Vice President Marketing

IMS CONSULTING (2011-2017)
Principal

BOOZ & COMPANY (2010-2011)
Principal

MONITOR GROUP (1994-2009)
Global Account Manager

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Pipeline: solid and advanced product portfolio in ophthalmic Gene Therapy

Technology	Product Candidate	Indication	Research	Preclinical	Phase I/II	Phase III	Registration	
MTS platform	LUMEVOQ™ (FDA & EMA Orphan Drug Designation)	**LHON ND4 (EU)**	●				→	**REVERSE**: Phase III top-line data reported in Apr (48w) & Oct (72w) 2018 and in May 2019 (96w)
		LHON ND4 (US)	●			→		**RESCUE**: Phase III top-line data reported in Feb (48w), Apr (72w) and Sep (96w) 2019 **REFLECT***: Phase III recruitment completed in July 2019, top-line data expected in Q1 2021
	GS011	**LHON ND1**	●	→				Initiate preclinical studies following GS010 Phase III clinical data
	Undisclosed Mitochondrial Target	Undisclosed	●	→				
Optogenetics	**GS030** (FDA & EMA Orphan Drug Designation)	**RP**	●		→			**PIONEER**: Second cohort treated in PIONEER Phase I/II clinical trial. Report interim data one year after last subject treated
	GS030	**Dry AMD & Geographic Atrophy**	●	→				

*Conducting this trial under a special protocol assessment with the FDA

Lead candidate, LUMEVOQ™, is expected to file for **MAA in Europe this year**

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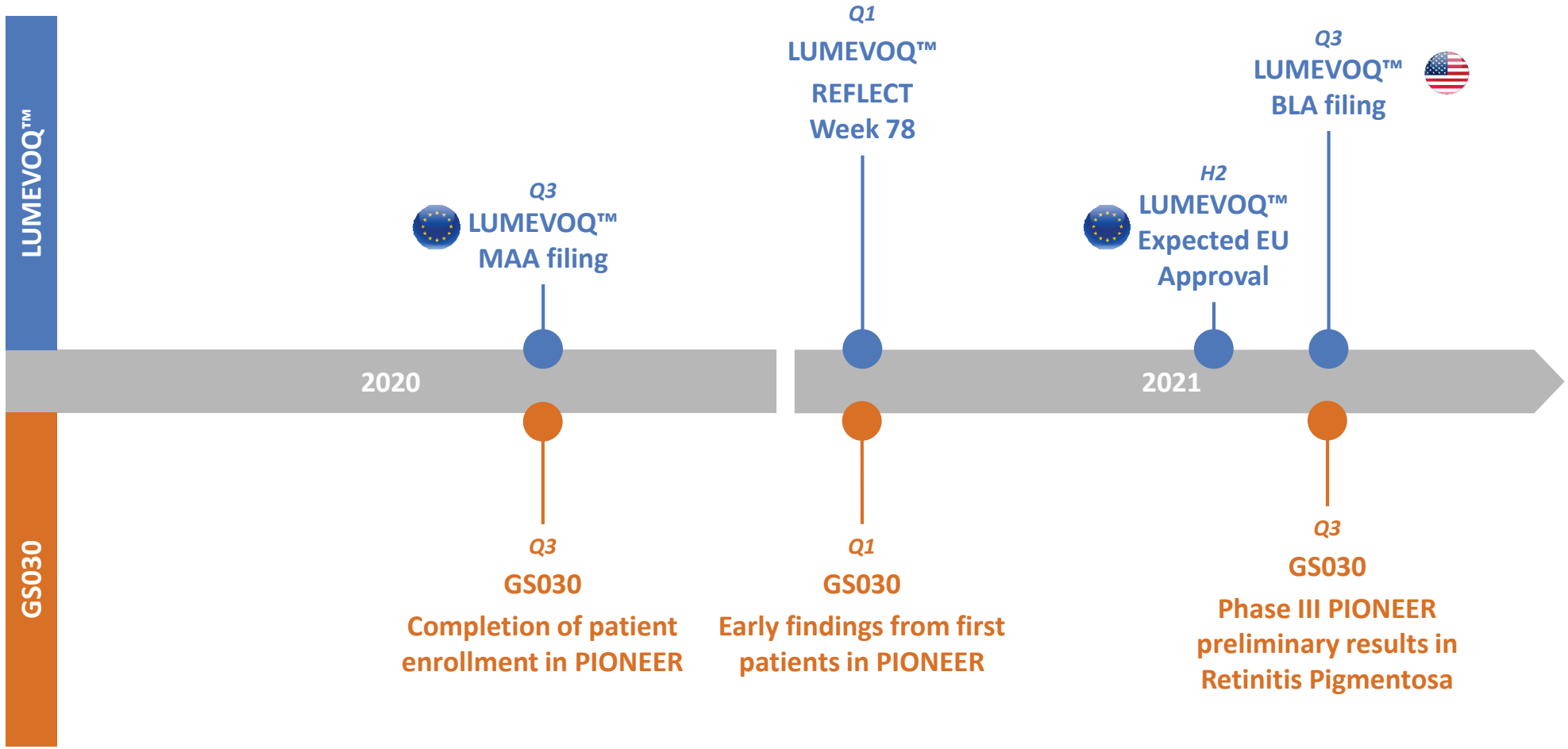
Rich upcoming news flow with numerous inflexion points



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LUMEVOQ™ (GS010) in LHON-ND4

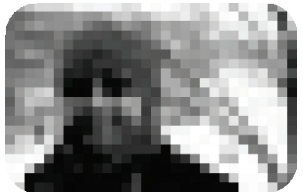
Last Phase III ongoing in Leber Hereditary Optic Neuropathy

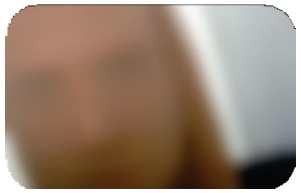
Commercial preparation ongoing for 2021 launch

Leber Hereditary Optic Neuropathy (LHON-ND4) high unmet medical need

What is LHON-ND4

- Rare inherited mitochondrial disease leading to degeneration of retinal ganglion cells (RGCs) and their axons, most often leading to **sudden loss of central vision**
- Sudden loss typically occurs at age 15-35, mostly in men
- **97%** of patients have bilateral involvement < 1 year / 25% of cases are simultaneous
- 90% of LHON patients have genes **MT-ND4 (~75% in US/EU)**, MT-ND1 and/or MT-ND6 affected





Incidence (new cases per year)	~800-1,200
Prevalence	~15,000-22,000

Progressive disease

- Rare recovery from vision **nadir**(1) reached during acute phase

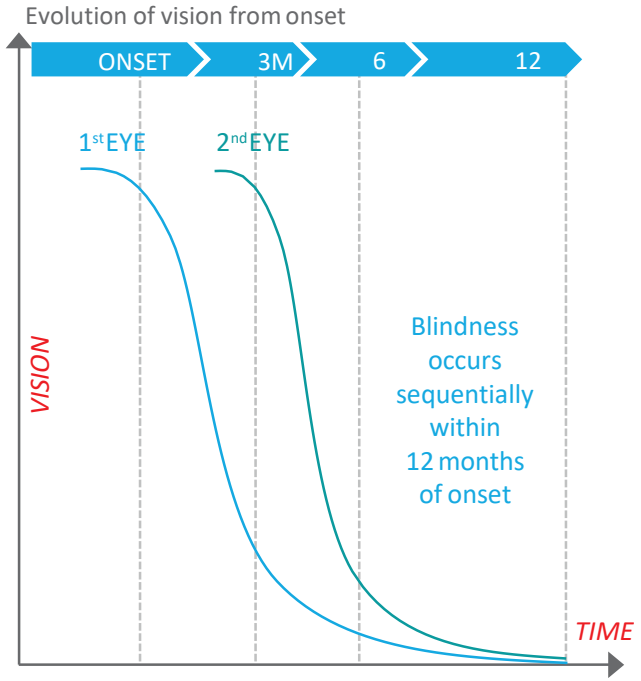


Image source: illustrated from Newamn NJ et al., Am J Ophthalmom. 141(6), 1061-1067,2006

Current treatment paradigm

- No cure for LHON-ND4
- Low-vision aids are primary supportive care
- Santhera's Raxone EU approved (under exceptional circumstances) in 2015 with mechanism of action partially relying on bypassing the dysfunctional complex I of the mitochondrial respiratory chain
  - Approved based on Phase 2 data, Phase 4 ongoing
  - Demonstrated **3 letters improvement** vs placebo (p=0.291 / NS) at week 24 in Best recovery of Visual Acuity (primary)(2)
  - Demonstrated **6 letters improvement** vs placebo (p=0.078 / NS) at week 24 in Change in best Visual Acuity(2)

(1) Nadir: worst visual acuity from baseline
(2) Raxone European full prescribing information https://www.ema.europa.eu/en/documents/product-information/raxone-epar-product-information_en.pdf

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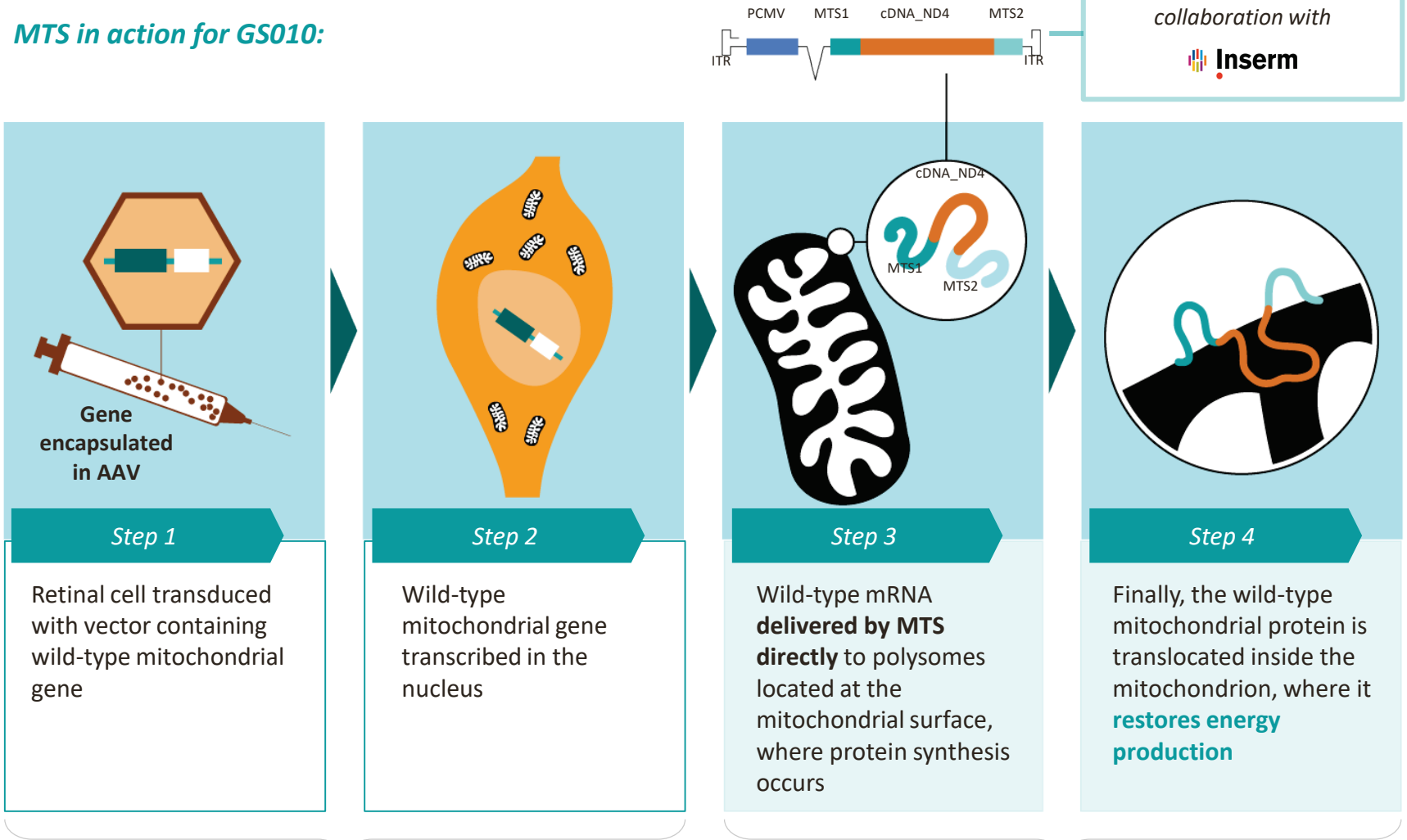
LUMEVOQ™ introduces Gene Therapy solution

Replacing affected mitochondrial mRNA via proprietary *MTS* technology*

The product of research collaboration with Inserm

MTS in action for GS010:



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RESCUE & REVERSE Phase III trials with unilateral injection demonstrated unprecedented improvement

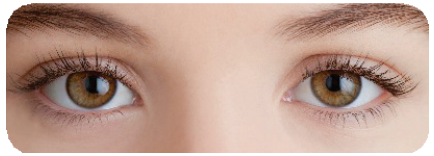
Different patient inclusion criteria | **Same design** | **Visual recovery at Week 96 and vs natural history**

REVERSE

- Onset of disease
  6 months to ≤ 1 year
- 37 patients enrolled

- Double-masked, multi-center
- One eye randomized to GS010; other eye received sham injection

Group 1



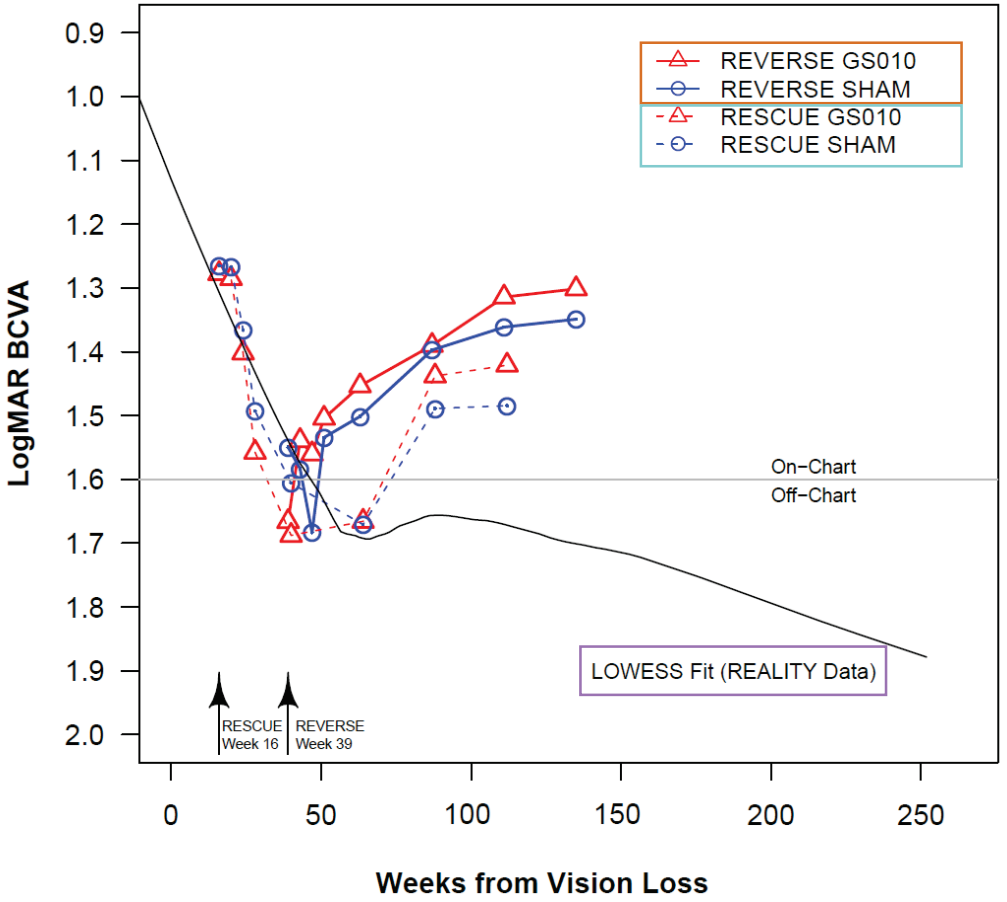
GS010 in right eye | **SHAM** in left eye

RESCUE

- Onset of disease
  ≤ 6 months
- 39 patients enrolled

Group 2

SHAM in right eye | **GS010** in left eye



REVERSE 6-12 MONTHS
+28 ETDRS Letters vs nadir

RESCUE 0-6 MONTHS
+26 ETDRS Letters vs nadir

REALITY LHON REGISTRY
Retrospective Natural History

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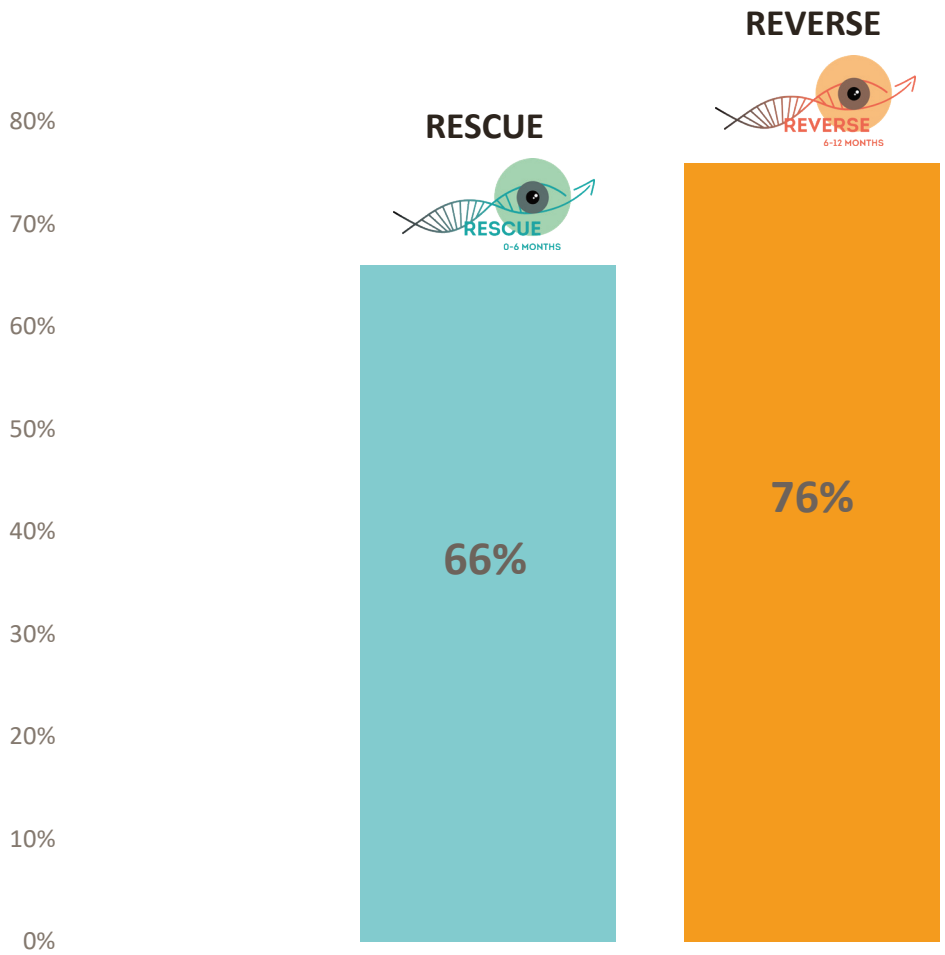
REVERSE and RESCUE demonstrate that over 2/3 of patients benefit from treatment



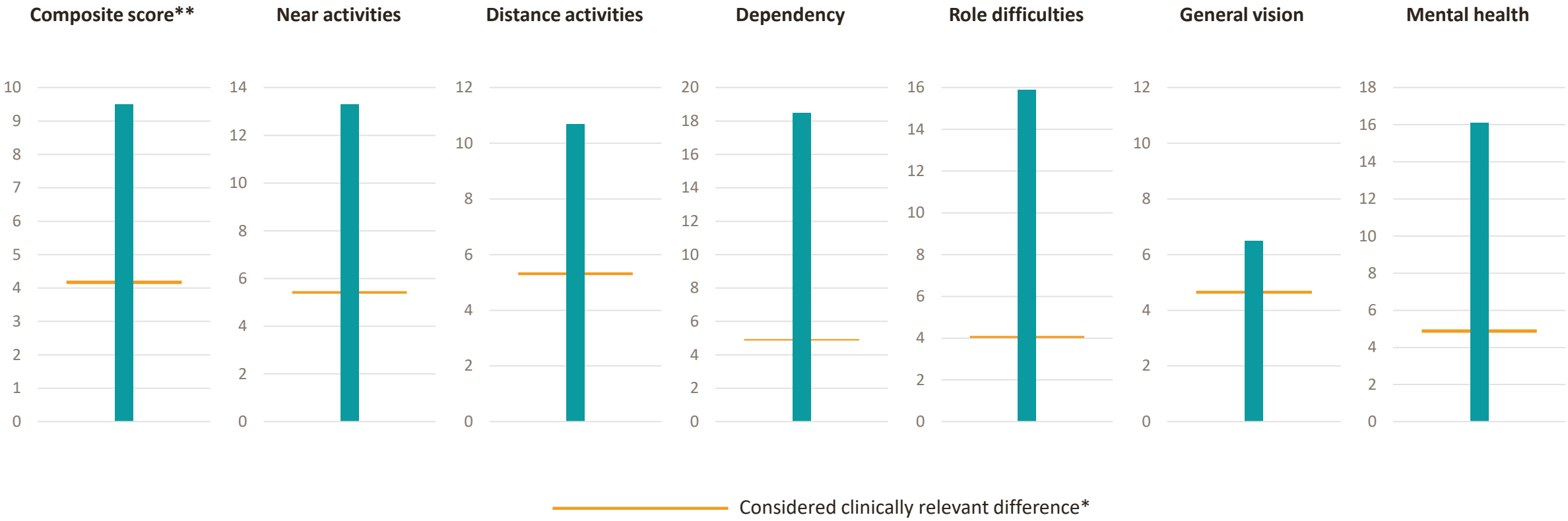
76% of REVERSE subjects achieved at least 15 letters improvement vs nadir in one or two eyes

66% of RESCUE subjects achieved at least 15 letters improvement vs nadir in one or two eyes

LUMEVOQ™ shows meaningful improvement on Quality of Life metrics

NEI VFQ-25 Results from REVERSE study

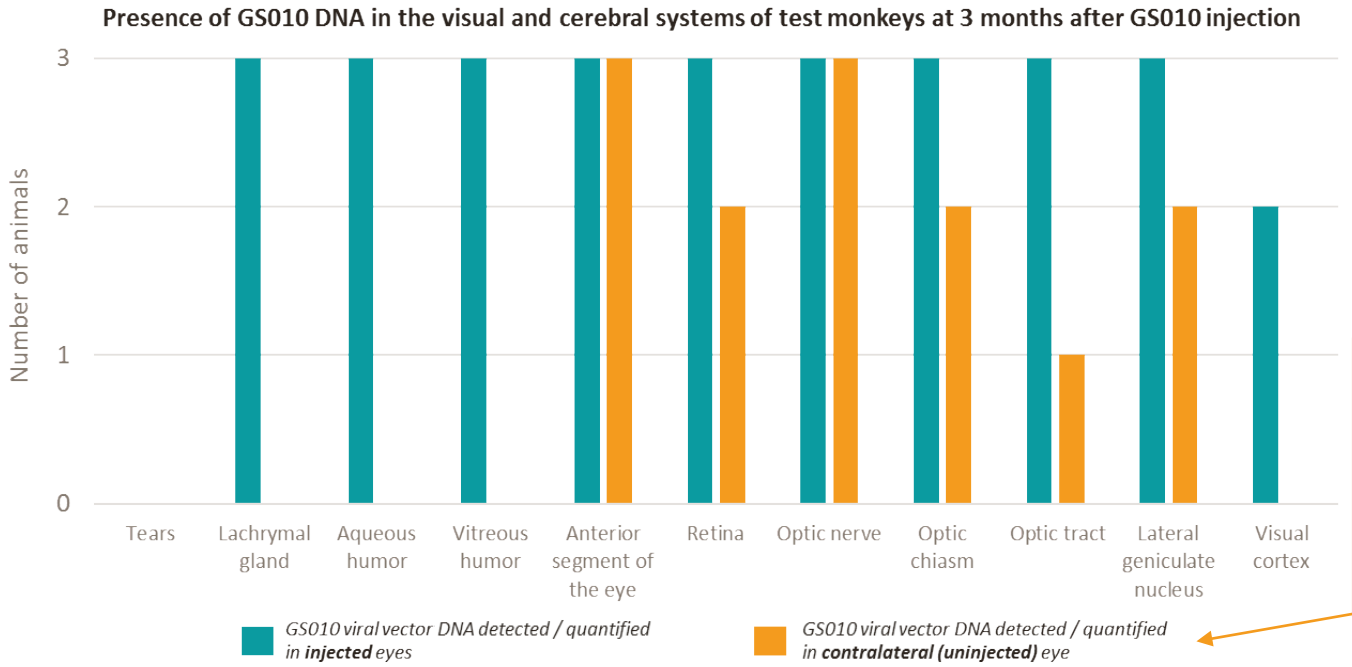
Mean change from baseline (absolute score) at week 96



***** Suñer *et al.* (2009): clinically relevant score differences based on a clinically significant 15-letter BCVA improvement at 12 months.
****** The composite score is an average of the vision-targeted sub-scale scores, excluding the general health rating question.

GS010 (LUMEVOQ™) viral vector DNA detection in uninjected eye of monkeys supports bilateral effect in REVERSE and RESCUE Phase III trials

Viral vector DNA detected in uninjected eye → potential mechanism for bilateral effect in REVERSE and RESCUE



- Three test monkeys injected in one eye using dose equivalent of treatment in REVERSE and RESCUE trials
- Highly sensitive validated test for presence of GS010 DNA used on tissue samples from primates in study
- **Key finding:**
  - **GS010 viral vector DNA was detected/quantified in many tissue samples from contralateral (uninjected) eye**

"The presence of viral vector DNA in the optic chiasm and optic nerve of the contralateral uninjected eye points towards a possible diffusion pathway."

Dr. Patrick Yu-Wai-Man, Senior Lecturer & Honorary Consultant Ophthalmologist at the University of Cambridge, Moorfields Eye Hospital, and the UCL Institute of Ophthalmology, London, UK

Notes: One control monkey was injected in one eye with saline solution. Three test monkeys were injected with GS010 in one eye using dose allometrically equivalent to that used in REVERSE and RESCUE. Tissue samples were taken at 3 months after injection and tested using a protocol that specifically targeted the CMV promoter of the GS010 DNA. The sensitivity, specificity and accuracy of the test were validated in a dedicated study.

LUMEVOQ™ safe and well-tolerated through week 96 in REVERSE & RESCUE Phase III studies

- LUMEVOQ™ was well-tolerated throughout both studies
- No serious adverse events in LUMEVOQ-treated eyes, and no discontinuation due to ocular events
- Most frequently seen ocular adverse events in the therapy group were mainly related to the injection procedure
- Occurrence of intraocular inflammation likely related to LUMEVOQ:
  - Accompanied by elevation of intraocular pressure in some patients “without any long-term sequelae”
  - Responsive to conventional treatment and without sequelae
- No systemic serious adverse events or discontinuations related to study treatment or study procedure

LUMEVOQ™ was well-tolerated through **96 weeks** after injection

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Last ongoing Phase III trial: REFLECT to assess efficacy and safety of bilateral injection

Double-masked, confirmatory study under Special Protocol Assessment from FDA

Q1 2021
LUMEVOQ™
REFLECT
Week 78
Read-out

REVERSE 6-12 MONTHS

RESCUE 0-6 MONTHS

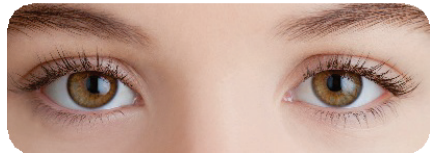
REFLECT BILATERAL

Patient inclusion criteria

- **98 patients** with vision loss ≤ 1 year
- **Initiation:** 4Q 2017 (1st patient treated in March 2018)
- Recruitment completed in July 2019

Design

Group 1



GS010 in first affected eye

GS010 in second affected eye

First affected eye always treated with GS010

Second affected eye randomized between GS010 and placebo

GS010 in first affected eye

Placebo in second affected eye

Group 2

Endpoints at Week 78

Primary

- Difference in change of vision compared to baseline between GS010 Treatment vs. Placebo in second affected/not yet affected eyes
  (LogMAR visual acuity used for statistical analysis)

Secondary

- Best-Corrected Visual Acuity at 2 years
- Spectral domain OCT biomarkers
- Humphrey visual field analysis
- Pelli-Robson Low Vision Contrast Sensitivity
- Quality of life assessments

Source: Company

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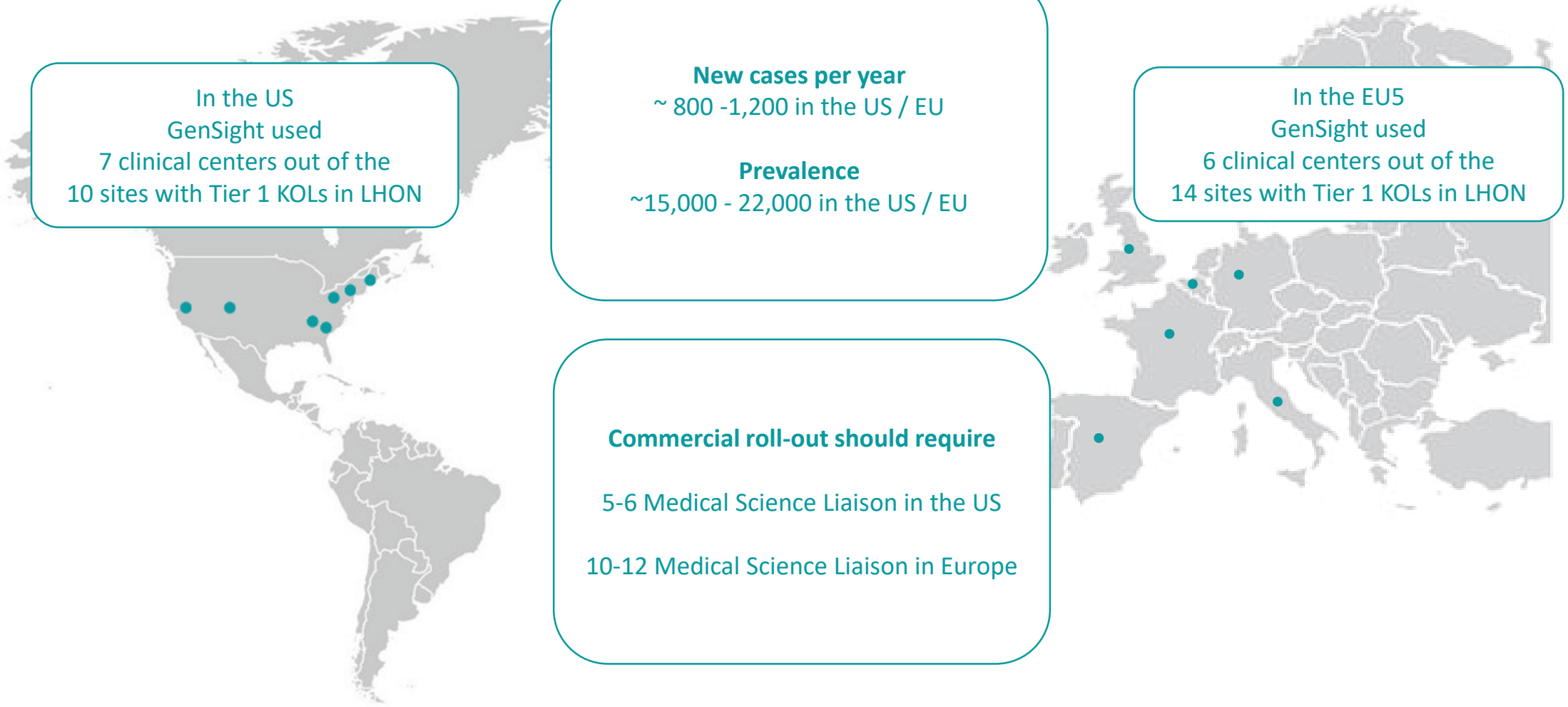
LHON is treated in just a few hyper-specialized centers, requiring limited commercial infrastructure and allowing proximity to the patients

In the US
GenSight used
7 clinical centers out of the
10 sites with Tier 1 KOLs in LHON

New cases per year
~ 800 -1,200 in the US / EU

Prevalence
~15,000 - 22,000 in the US / EU

In the EU5
GenSight used
6 clinical centers out of the
14 sites with Tier 1 KOLs in LHON



Commercial roll-out should require

5-6 Medical Science Liaison in the US

10-12 Medical Science Liaison in Europe

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Manufacturing strategy validated

Top quality toll manufacturer selected allowing:

- US based manufacturing
- Leverage manufacturing expertise and ability to scale up
- Lower production risk
- Reduce regulatory risk
- Increase flexibility
- No capex
- Optimize gross margin

Limited number of copies required per injection means:

- 5-6 manufacturing batches per year sufficient to treat EU/US expected demand
- 36-month shelf life provides flexibility to adjust to demand fluctuations

Planning for manufacturing redundancy to further reduce manufacturing risk

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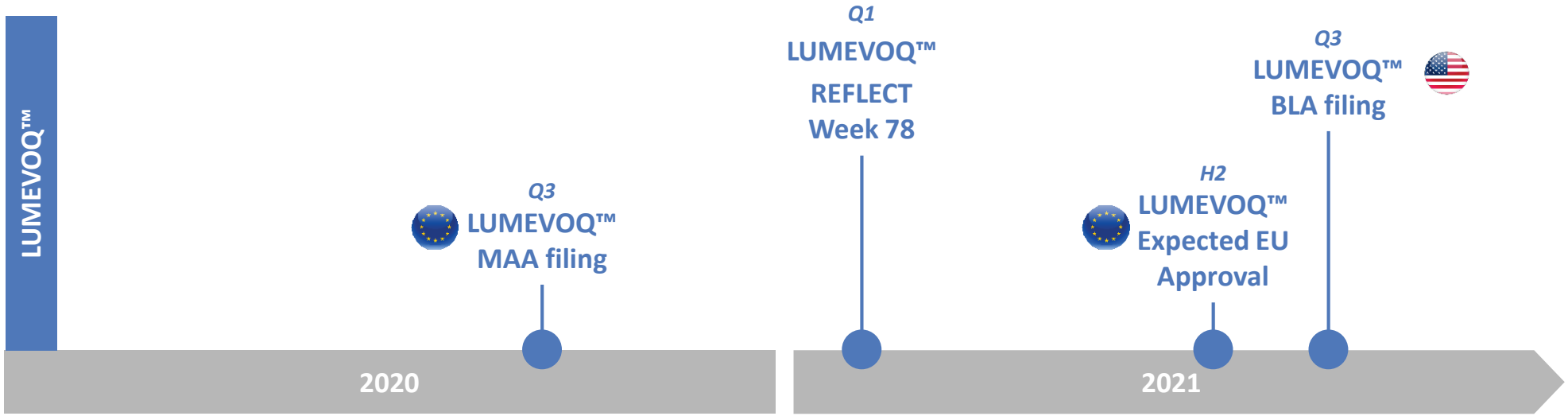
LUMEVOQ™ Key milestones



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Compassionate Use for LUMEVOQ™ (GS010)

Seeking use of an investigational medication under circumstances a patient may not be able to participate in a clinical trial and before MA/BLA approval by regulatory authorities

- Individual patients Expanded Access INDs have been approved by the FDA for GS010 (lenadogene nolparvovec)

- *"ATU Nominative"* - named patient Temporary Authorization for Use - for LUMEVOQ™ granted by ANSM to CHNO of the *Quinze-Vingts* in Paris
- Bilateral injections priced at €700,000 per patient, expected to generate revenues in 2020
  - Reimbursement warranted by the national Social Security up to € 30M/year
- Next step : seeking for a Cohort ATU *"ATU de Cohorte"*

GS030

Second product candidate targeting photoreceptor degenerative diseases:

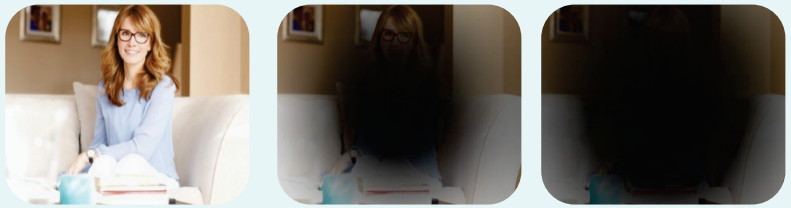
- Retinitis Pigmentosa (RP)
- Age-Related Macular Degeneration (AMD)

Treating the 2 main degenerative diseases of photoreceptors that lead to blindness

Retinitis Pigmentosa

- Blinding genetic disease caused by mutations in over 100 different genes
- Sequential photoreceptor degeneration leads to slow & irreversible progression to blindness, usually at age 40-45
- 15-20,000 new patients each year in the US and EU

Geographic Atrophy (GA) in AMD (Age-Related Macular Degeneration)



- Early (dry-form) AMD evolves with age into late AMD, one of whose forms is GA
- Dry-AMD affects 350-400,000 new patients a year
- Prevalence of GA increases with age, from 3.5% among 75-year-olds to 22% among those over 90
- Late AMD patients with GA account for 10-20% of blind patients in their age group

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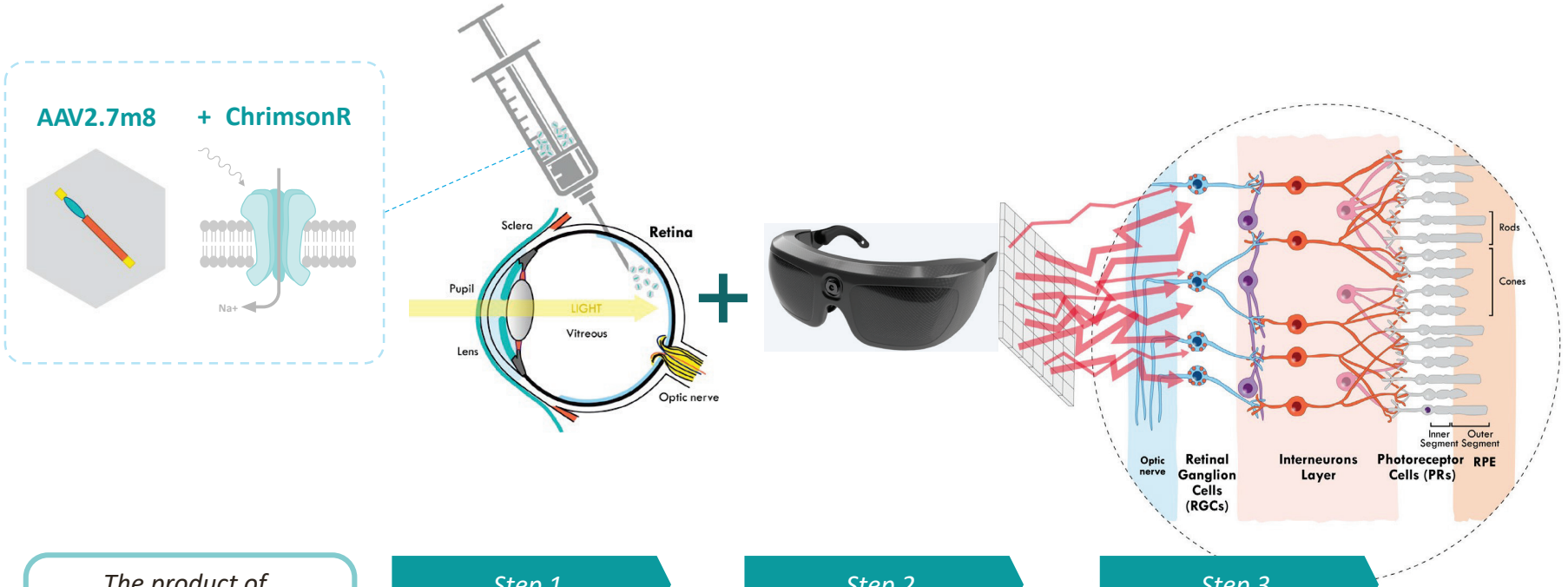
GS030: using Gene Therapy to rejuvenate production of light-sensitive protein and restore vision



The product of research collaboration with

Massachusetts Institute of Technology

ADVERUM BIOTECHNOLOGIES

UPMC

Step 1

Gene Therapy transfer of the gene that encodes light-sensitive protein **Expression** in retinal ganglion cells (RGCs)

Step 2

Stimulation with **optoelectronic device** to transform external light stimuli into signal that can activate the RGCs

Step 3

Retinal output sent to brain for image processing

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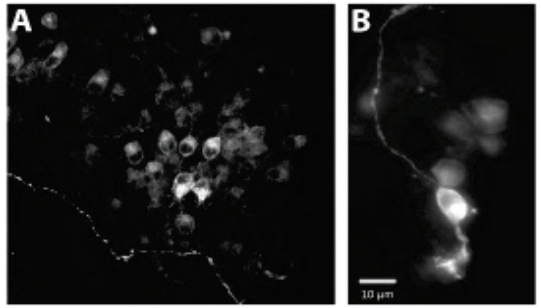
GS030 leads to functional vision restoration in monkey and rats

Localization of light-sensitive protein in NHP retina

Expression of ChR-tdT in midget cells of monkey perifovea

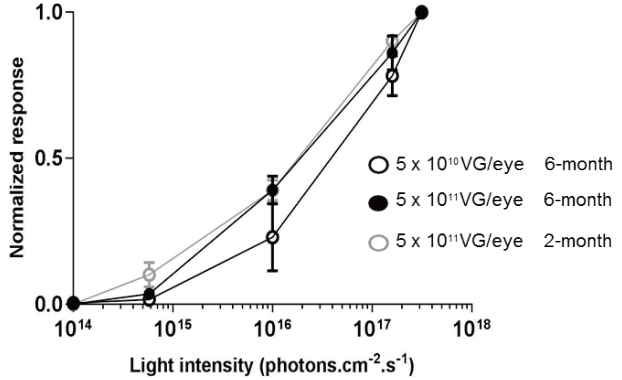
In vivo in NHP assessment 6 months after IVT injection



Dose-ranging response to firing relationship in NHP

Active dose range : 5x10^{10} and 5x10^{11} VG/eye

MEA assessment 6 months after IVT injection in NHP

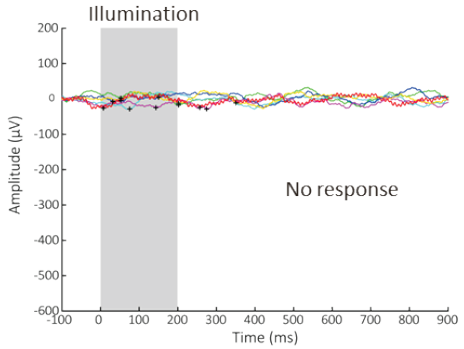


Restoration of a functional vision in P23H rats

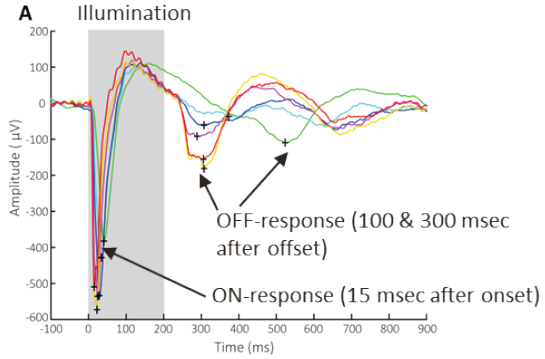
Light-induced visual evoked cortical responses

Full field 590 nm light from ~ 4.7x10^{15} to 1.1x10^{17} photons/cm^2/sec

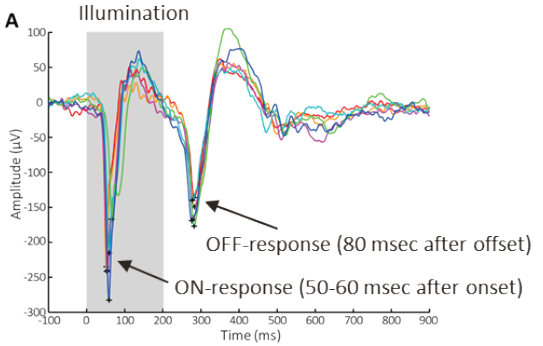
Untreated P23H rat



GS030-treated P23H rat



Normal Long-Evans rat



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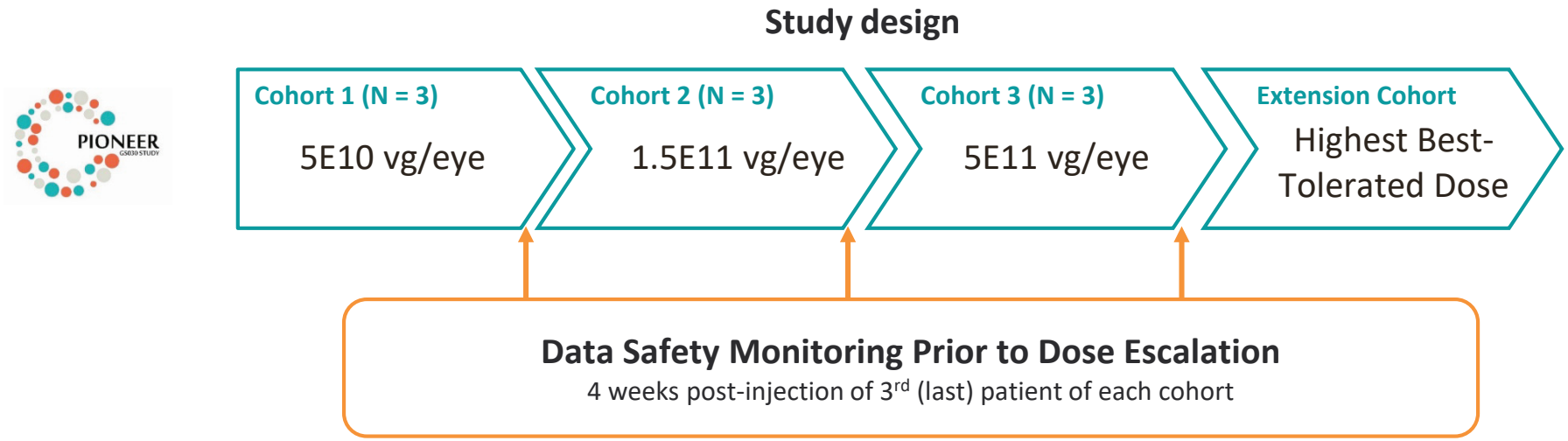
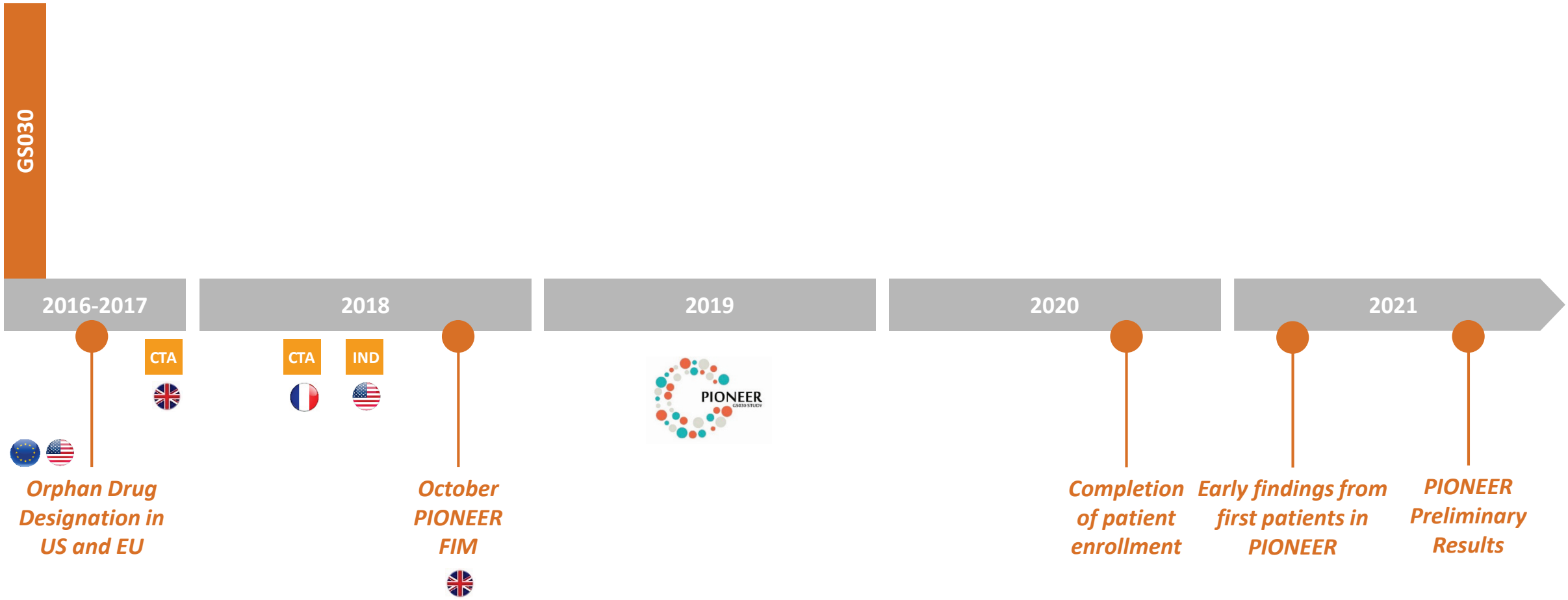
PIONEER Phase I/II clinical trial: A First-in-Man study



- **First-in-man**, dose-escalation safety study, multi-center (France, UK, US)
- **Study population:** end-stage non-syndromic RP (vision < Counting Fingers)
- **Primary analysis:** Safety at 1 year
- Single intra-vitreal injection in the **worst affected** eye
- Decision to increase the dose taken by a DSMB

2nd cohort fully enrolled and treated

GS030 Key Milestones



Building high strategic value

A company developing innovative and versatile technology platforms nearing commercialization and evolving in an area where value is increasingly being recognized by the market

GenSight at the forefront of Gene Therapy with potential product launch in 2021

» **LUMEVOQ™ in LHON-ND4**
- Strong clinical data
- Upcoming confirmatory Phase III trial

» **Targets attractive market**
- High unmet medical need
- Virtually no competition
- Well defined path to commercial success

» **Proprietary MTS technology**
- Broad range of mitochondrial diseases

» **Rich news flow** in 2020 and 2021

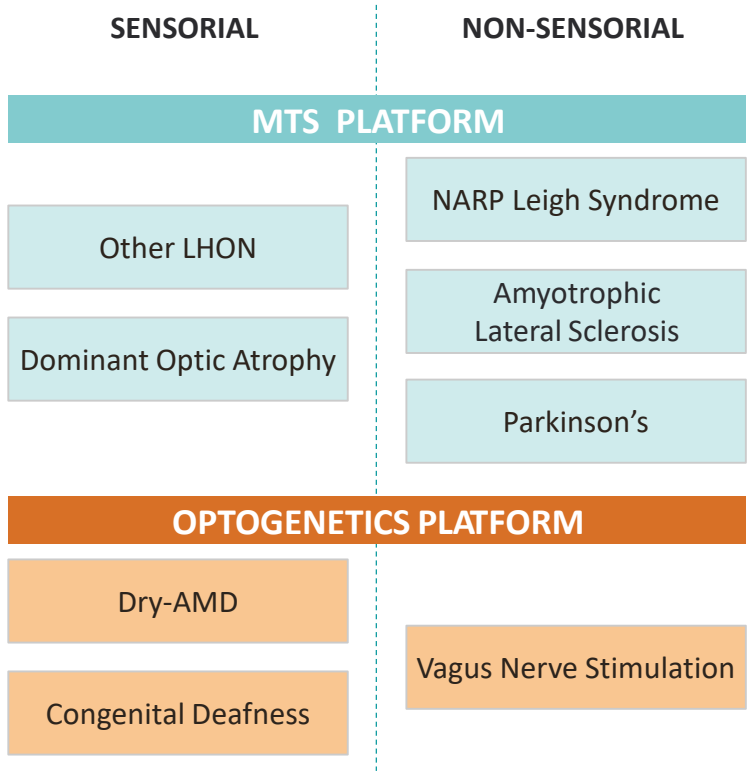
Gene Therapy increasingly attracts interest from investors and Large Pharma

» **Viable therapeutic option** (already 3 approved therapies)

» **Pricing reflective of significant therapeutic benefit**

» **Large Pharma increasingly involved in the field**

LUMEVOQ™ and Beyond: Two platforms pargeting large number of sensorial and non-sensorial diseases



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GenSight Biologics in numbers

Key financial information

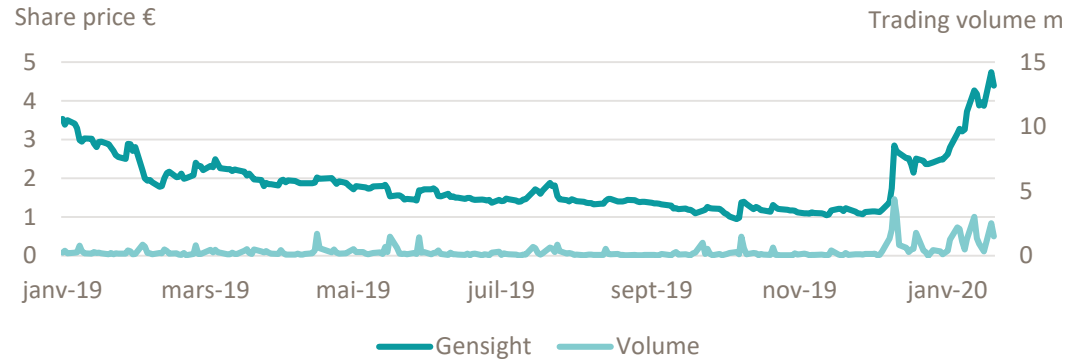
Company Overview	
Market Cap*:	€ 119m
Cash Position (Dec 31, 2019):	€ 19.2m
Outstanding Shares:	32.8m
Latest Amount Raised (Dec 20, 2019):	€ 15m
Raised to date	€ 142m
IPO Date	July 2016

Analyst Coverage

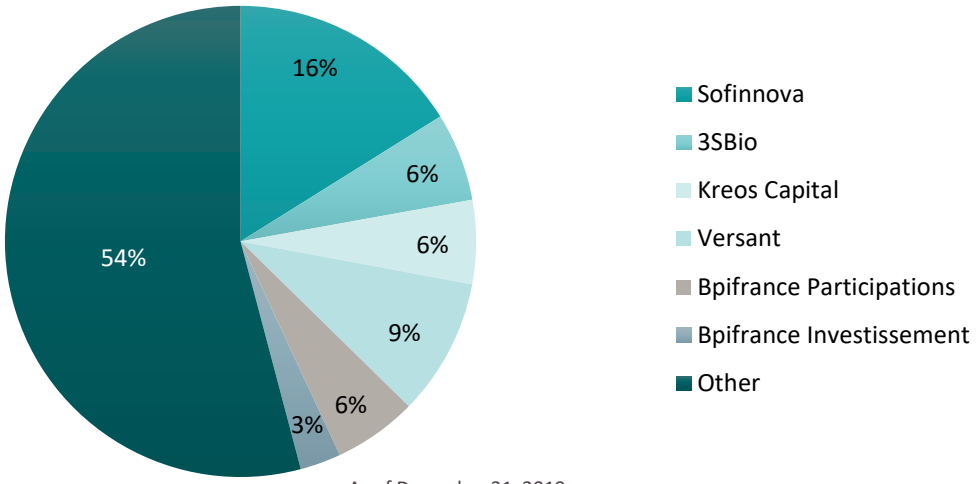
- Oddo & Cie: Martial Descoutures (FR)
- Gilbert Dupont: Jamila El Bougrini (FR)
- Chardan: Gbola Amusa (US)
- NIBC: Dylan van Haaften (NL)

*As of December 31, 2019

Share price evolution and trading volume



Shareholder structure



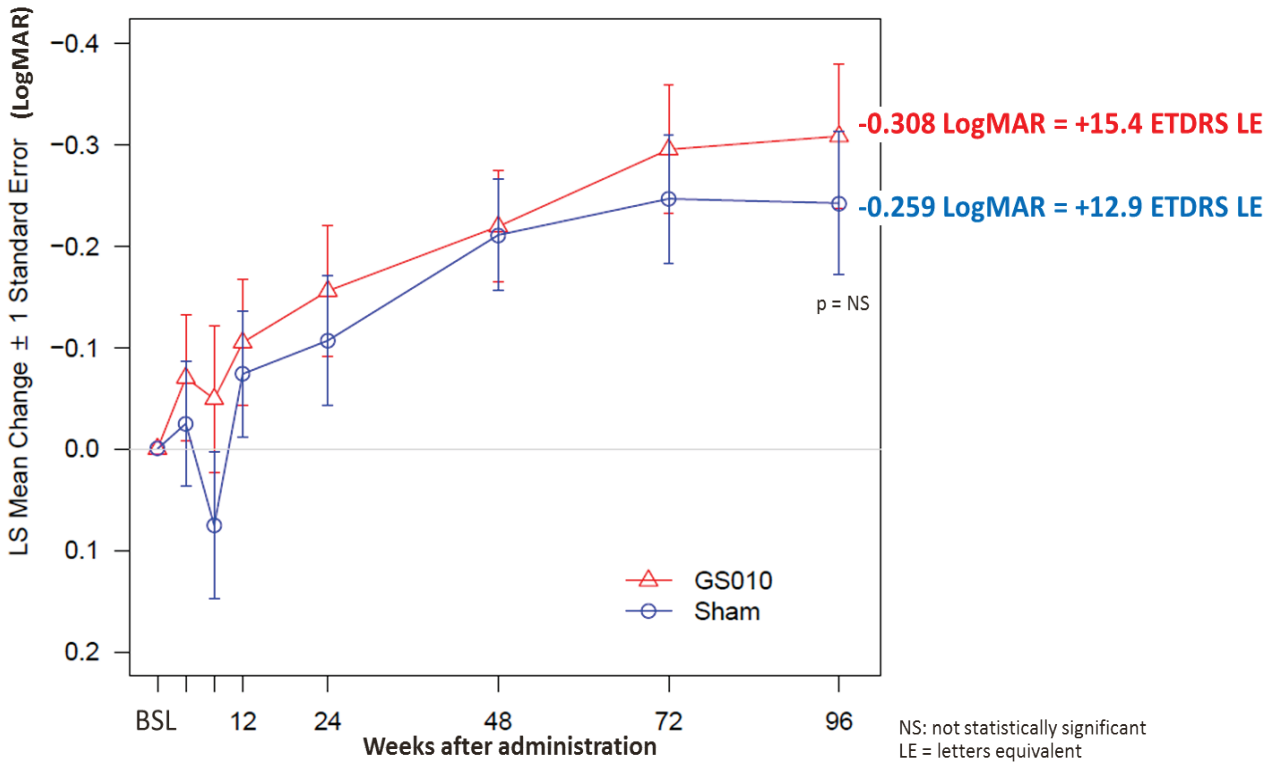
As of December 31, 2019

Corporate calendar	Date
2019 Full-Year Financial Update and Statements	March 12, 2020
2020 1Q Cash Position	April 21, 2020
Annual General Meeting	April 29, 2020
2020 First-Half Financial Update and Statements	July 30, 2020
2020 3Q Cash Position	October 20, 2020
2020 4Q Cash Position	January 19, 2021

Back-up Slides

Visual Acuity: REVERSE 96-week

Visual Acuity bilaterally improved by +15 and +13 ETDRS letters equivalent from baseline to Week 96 in GS010- and sham-treated eyes, respectively, sustaining the gain at Week 72

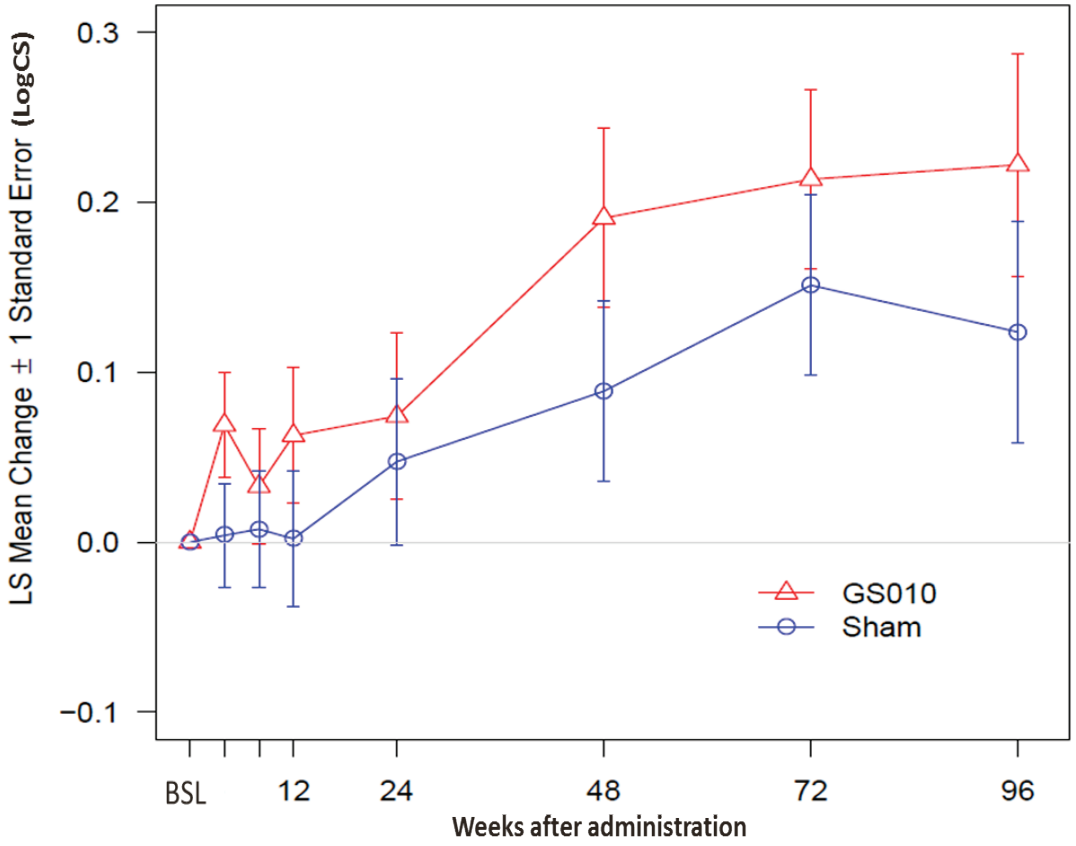


NS: not statistically significant
LE = letters equivalent

Mean visual acuity (BCVA) among GS010-treated eyes and sham-treated eyes evolved with similar trajectories, worsening to a post-treatment low point, or nadir, before recovering at Week 96 by +28 and +24 ETDRS letters equivalent, respectively

Contrast Sensitivity: REVERSE 96-week

Like BCVA, contrast sensitivity (Pelli-Robson) showed a bilateral trend, improving from baseline to Week 96 in both GS010-treated and sham eyes



Mean contrast sensitivity for GS010-treated eyes showed a more robust improvement versus baseline over the course of the trial comparing to sham-treated eyes

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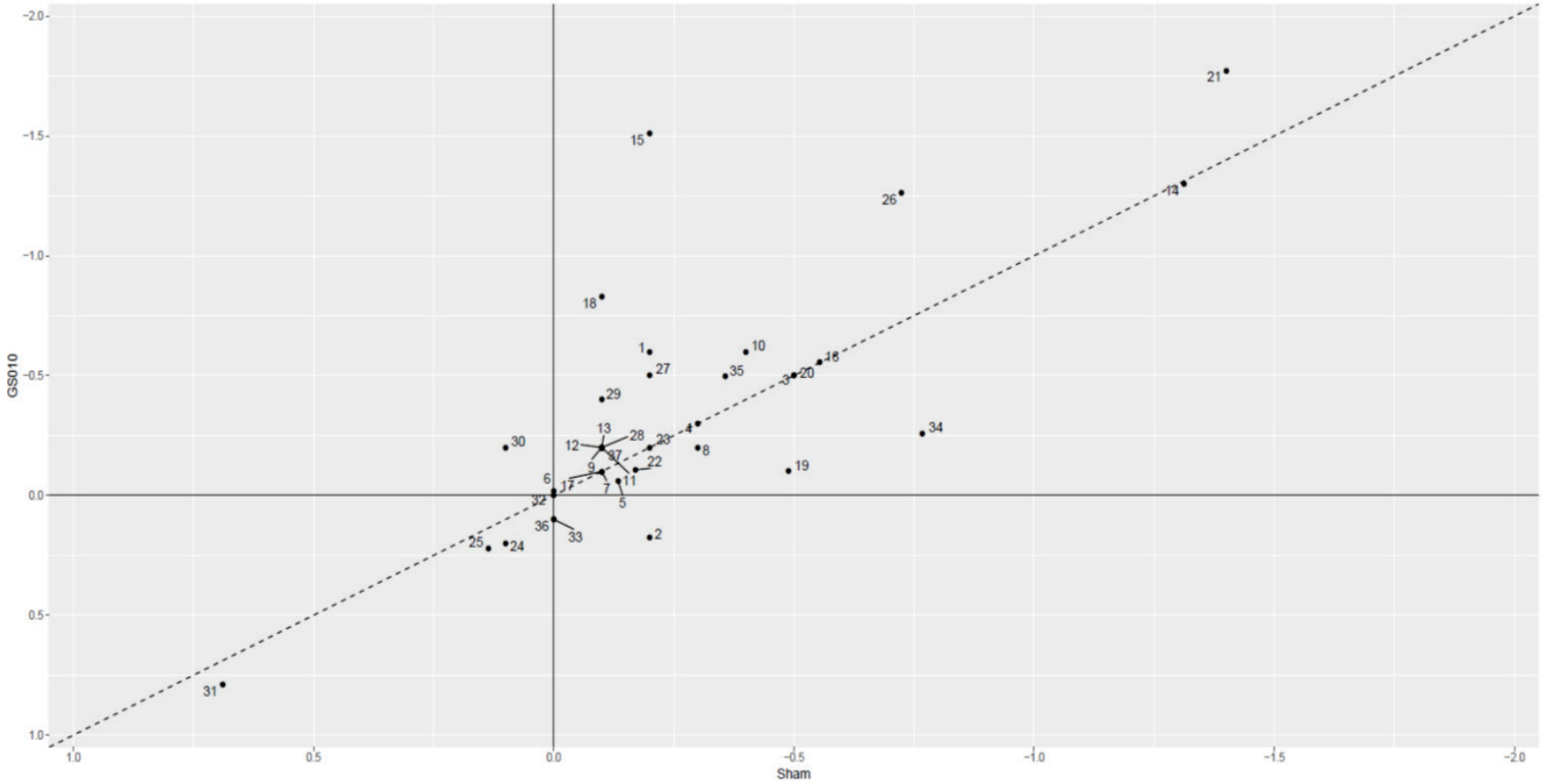
Responder Analysis: Subjects Mapping in REVERSE at 96 weeks

Visual Acuity Change of Sham eyes was correlated with that of GS010 eyes in most REVERSE subjects
Subjects are well concentrated around the mean

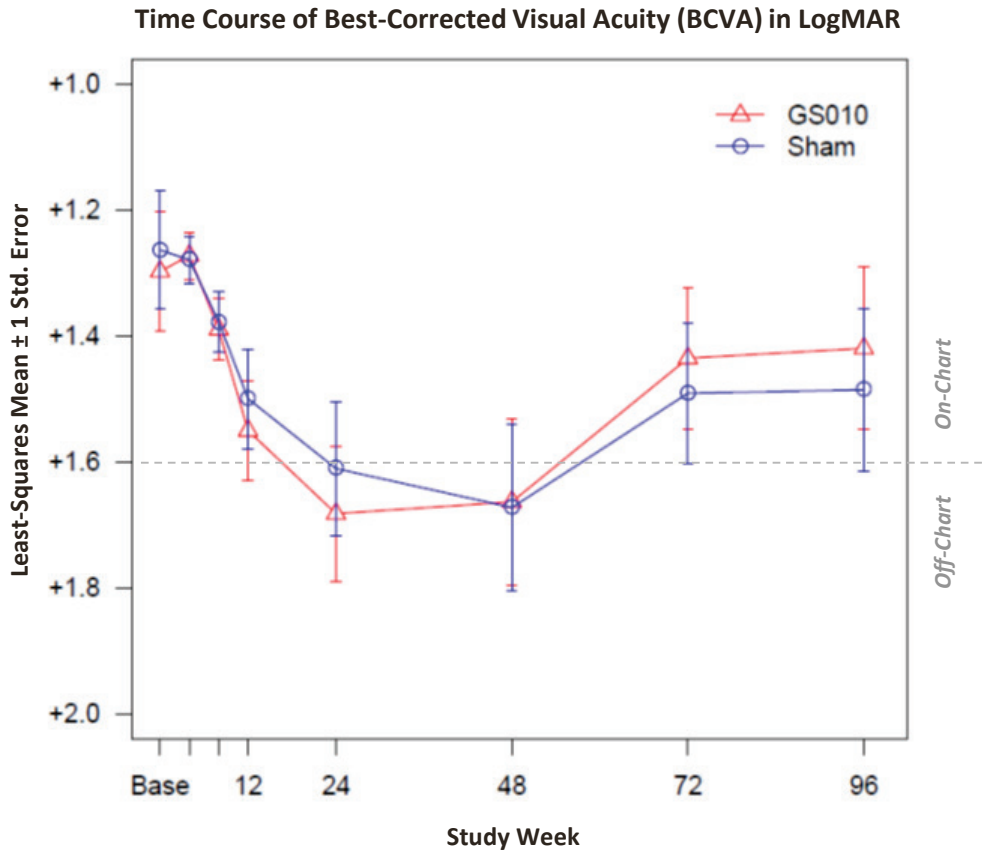
REVERSE – Scatterplot of Change from Baseline at Week 96

Pearson Corr Coeff = 0.776; p – 1.677^{e}-08 / Spearman Corr Coeff = 0.727; p = 3.492^{e}-07



Visual Acuity: RESCUE 96-week

Visual Acuity bilaterally improved by +26 and +23 ETDRS letters from nadir to week 96 in GS010- and sham-treated eyes, respectively. Mean visual acuity has transitioned from off-chart to on-chart.



Mean visual acuity (BCVA) among GS010-treated eyes and sham-treated eyes evolved with similar trajectories, worsening to a lowest point, or nadir, before significantly improving to week 96 – coherent with REVERSE

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Visual Acuity: Improvement from Baseline

REVERSE: Continuous bilateral improvement of Visual Acuity up to Week 96
RESCUE: Worsening VA compared to baseline reflects brutal progression of LHON

LS Mean (SE) [a]	Change from BASELINE					
	Week 48			Week 96		
	n	LogMAR	ETDRS Letters Equivalent	n	LogMAR	ETDRS Letters Equivalent
GS010 Eyes	37	-0.218 (0.055)	**+11**	37	-0.308 (0.068)	**+15**
Sham Eyes	37	-0.211 (0.055)	**+11**	37	-0.259 (0.068)	**+13**

LS Mean (SE) [a]	Week 48			Week 96		
	n	LogMAR	ETDRS Letters Equivalent	n	LogMAR	ETDRS Letters Equivalent
GS010 Eyes	38	+0.380 (0.129)	**-19**	38	+0.178 (0.120)	**-9**
Sham Eyes	38	+0.392 (0.129)	**-20**	38	+0.207 (0.120)	**-10**

[a] Efficacy Endpoint was assessed using a mixed model of analysis of covariance (ANCOVA), with change from baseline at week of interest as the response, and subject, eyes of the subject as random factor, treatment and the baseline LogMAR value as covariates.
Missing data were not imputed.

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Visual Acuity: Recovery from Nadir

Visual Acuity deteriorates to a low point before recovering significantly in both eyes

REVERSE 6-12 MONTHS	Change from NADIR [a]					
	Week 48			Week 96		
Mean (SD) [b]	n	LogMAR	ETDRS Letters Equivalent	n	LogMAR	ETDRS Letters Equivalent
GS010 Eyes	37	-0.553 (0.444)	**+27.6**	37	-0.566 (0.450)	**+28.3**
Sham Eyes	37	-0.478 (0.498)	**+23.9**	37	-0.490 (0.480)	**+24.5**

RESCUE 0-6 MONTHS	Week 48			Week 96		
Mean (SD) [b]	n	LogMAR	ETDRS Letters Equivalent	n	LogMAR	ETDRS Letters Equivalent
GS010 Eyes	34	-0.509 (0.496)	**+25.4**	34	-0.526 (0.479)	**+26.3**
Sham Eyes	33	-0.452 (0.495)	**+22.6**	34	-0.457 (0.485)	**+22.8**

[a] NADIR: Nadir was defined as the **lowest Visual Acuity** value **from baseline** up to Week of interest. LP/NLP vision was assigned a LogMAR value of 4.0 and 4.5 respectively.
[b] Mean change from nadir was calculated using observed values (no data were imputed).

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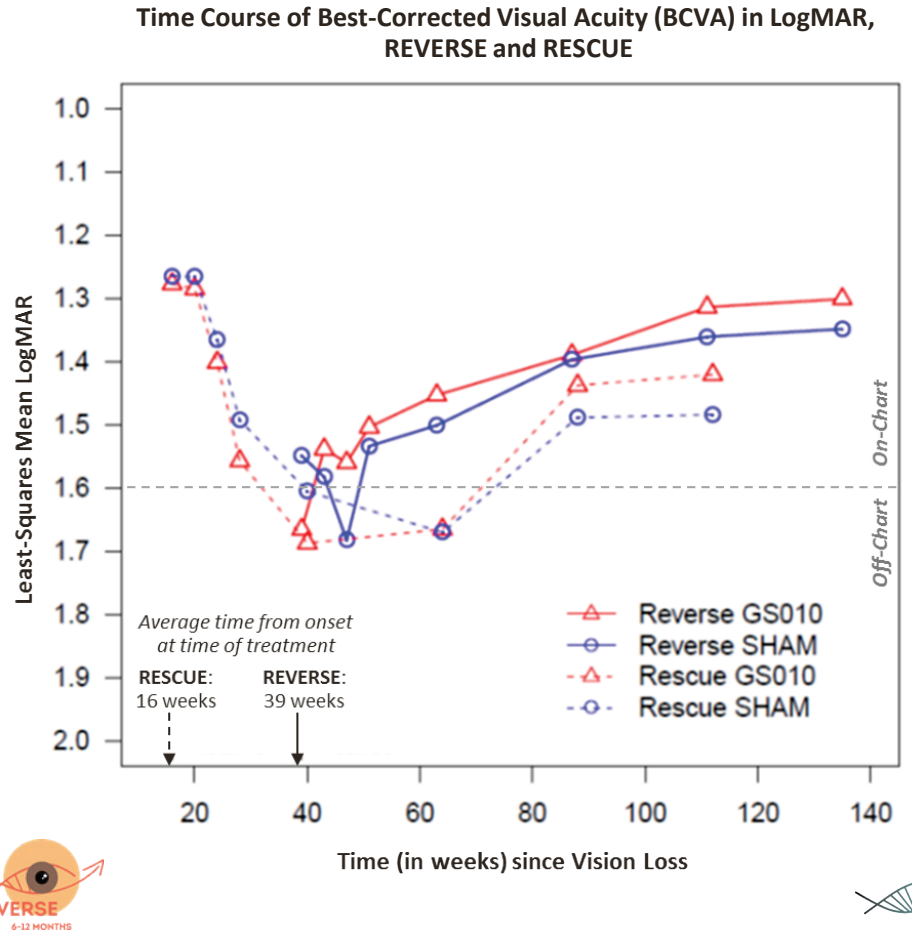
Visual Acuity: Time Course in LogMAR values in REVERSE and RESCUE

REVERSE and RESCUE show coherent pattern of meaningful and durable bilateral visual recovery from nadir



LogMAR All eyes Baseline	GS010 1.67 (0.50)	Sham 1.55 (0.42)	All 1.61 (0.46)

LogMAR All eyes Baseline	GS010 1.31 (0.52)	Sham 1.27 (0.62)	All 1.29 (0.57)

LUMEVOQ™ shows meaningful and sustained improvement on Quality of Life

- Composite score and relevant sub-scores in the National Eye Institute Visual Function Questionnaire-25 (NEI VFQ-25) showed sustained improvements versus baseline at Week 48, Week 72 and Week 96
- Magnitudes of mean score improvement observed with GS010 correlate with clinically meaningful improvements in best-corrected visual acuity (BCVA)

NEI VFQ-25 Results from REVERSE study

Mean change from baseline (absolute score and percent)

	Composite Score**	Near Activities	Distance Activities	Dependency	Role Difficulties	General Vision	Mental Health
Week 48	+7.2 *+23.2%*	+10.4 *+65.1%*	+9.6 *+49.8%*	+12.4 *+100.6%*	+14.5 *+65.0%*	+10.3 *+50.9%*	+11.2 *+81.9%*
Week 72	+8.1 *+25.2%*	+9.5 *+58.1%*	+8.2 *+42.5%*	+18.9 *+130.2%*	+15.2 *+70.9%*	+11.9 *+54.1%*	+15.2 *+105.6%*
Week 96	+9.5 *+28.8%*	+13.3 *+78.1%*	+10.7 *+47.4%*	+18.5 *+130.2%*	+15.9 *+78.9%*	+6.5 *+32.4%*	+16.1 *+108.2%*
Clinically relevant difference*	+3.90 to +4.34	+4.67 to +6.06	+5.15 to +5.38	+4.72 to +4.98	+3.31 to +4.70	+4.38 to +4.82	+4.70 to +4.88

*Suñer *et al.* (2009): clinically relevant score differences based on a clinically significant 15-letter BCVA improvement at 12 months.
**The composite score is an average of the vision-targeted sub-scale scores, excluding the general health rating question.

Our target: degenerative retinal diseases with underlying genetic causes

LEBER HEREDITARY OPTIC NEUROPATHY (LHON)

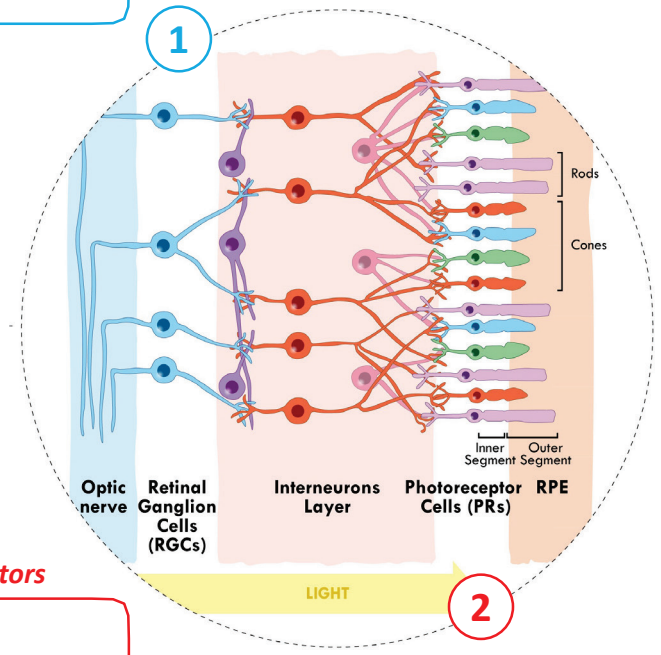
1. Degeneration of RGCs

Genetic mutations
Aging

2. Degeneration of photoreceptors

RETINITIS PIGMENTOSA (RP)

GEOGRAPHIC ATROPHY
(Late stage form of Age-Related Macular Degeneration - AMD)



✓ **Unmet need: high**

- Inexorable progression to blindness for most patients
- No approved treatments*

✓ **The eye: an ideal laboratory**

- Immune-privileged, closed system
- Intravitreal injections to introduce of genetic material close to target cells
- Slow turnover of retinal cells support long-term expression of transduced genes

✓ **AAV: proven vector for Gene Therapy**

- Proven safety and proof of effect in humans
- Efficient transduction of retinal cells
- No need to screen patients for Nab before treatment
- Validated manufacturing process

*Except for exceptional circumstances for idebenone in Europe

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Efficacy key findings: REVERSE & RESCUE

REVERSE: 96-Week Follow-Up

- Sustained bilateral improvement in visual acuity (BCVA) at Week 96
- Versus baseline: +15 ETDRS letters equivalent in GS010 eyes and +13 ETDRS letters equivalent in sham eyes
- Versus nadir: +28 ETDRS letters equivalent in GS010 eyes and +24 ETDRS letters equivalent in sham eyes
- 78% of REVERSE subjects attained Clinically Relevant Recovery (CRR) from nadir in one or two eyes, compared to 28% in a natural history study
- **76% of REVERSE subjects** achieved at least 15 letters improvement vs nadir in one or two eyes
- Patients' quality of life scores continue to increase, especially in ability to carry out vision-related activities
- Preservation of anatomy for both eyes, as observed for retinal layers of interest: GCL, Temporal and PMB RNFL

RESCUE: 96-Week Follow-Up

- Sustained bilateral improvement in BCVA from Week 48 to Week 96
  - From Week 48 to Week 96: +10 ETDRS letters equivalent in GS010 and sham eyes
- Compelling bilateral improvement in BCVA from Nadir
  - Versus nadir: +26 ETDRS letters equivalent in GS010 eyes and +23 ETDRS letters equivalent in sham eyes
- Clinically Relevant Recovery (CRR) from Nadir
  - 63% RESCUE subjects attained CRR in one or two eyes
  - compared to 28% in a natural history study
- **66% of RESCUE subjects** achieved at least 15 letters improvement vs nadir in one or two eyes
- Preservation of anatomy for both eyes, as observed for retinal layers of interest: GCL, Temporal and PMB RNFL

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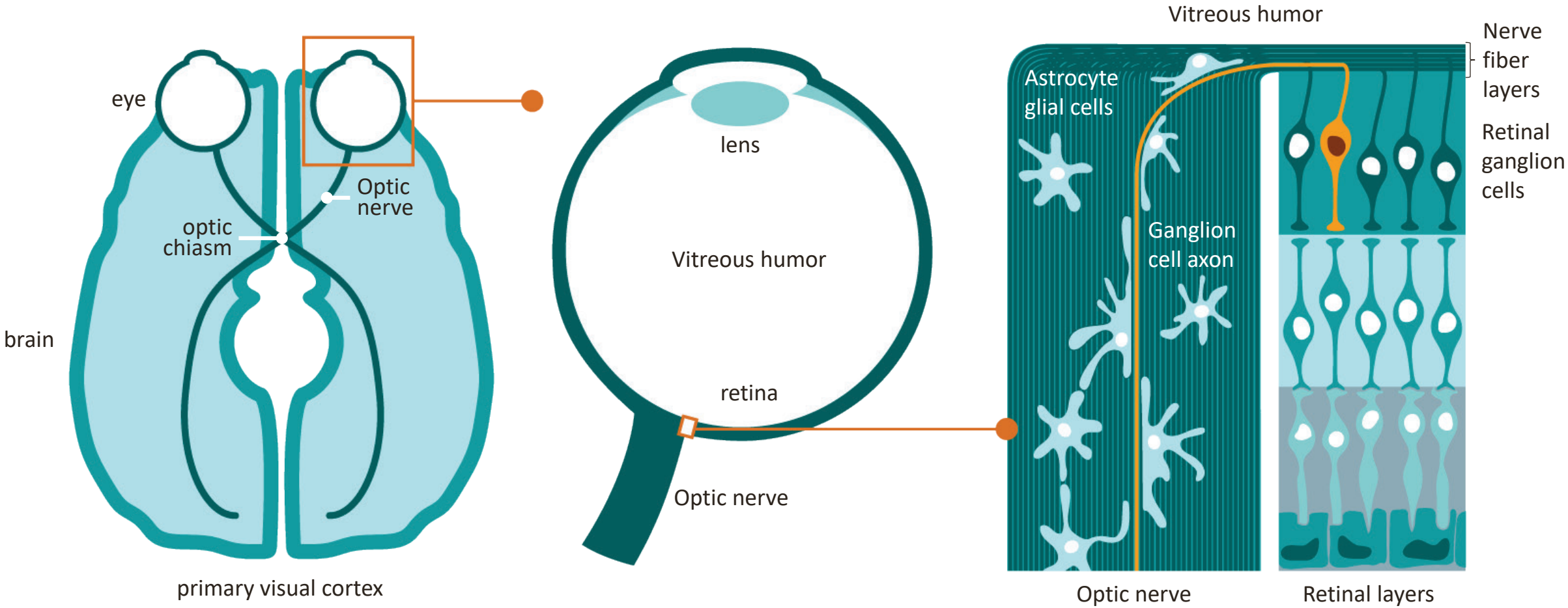
Brain and eye anatomy



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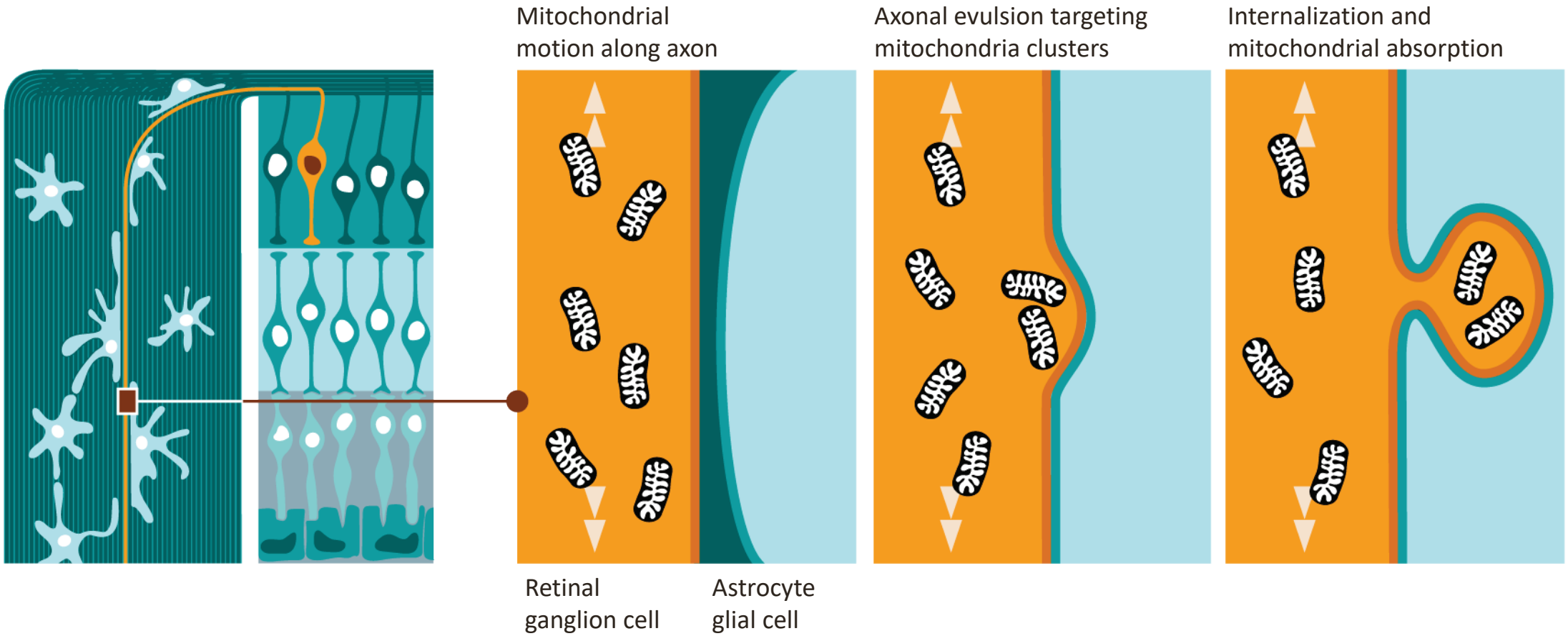
Mitophagy



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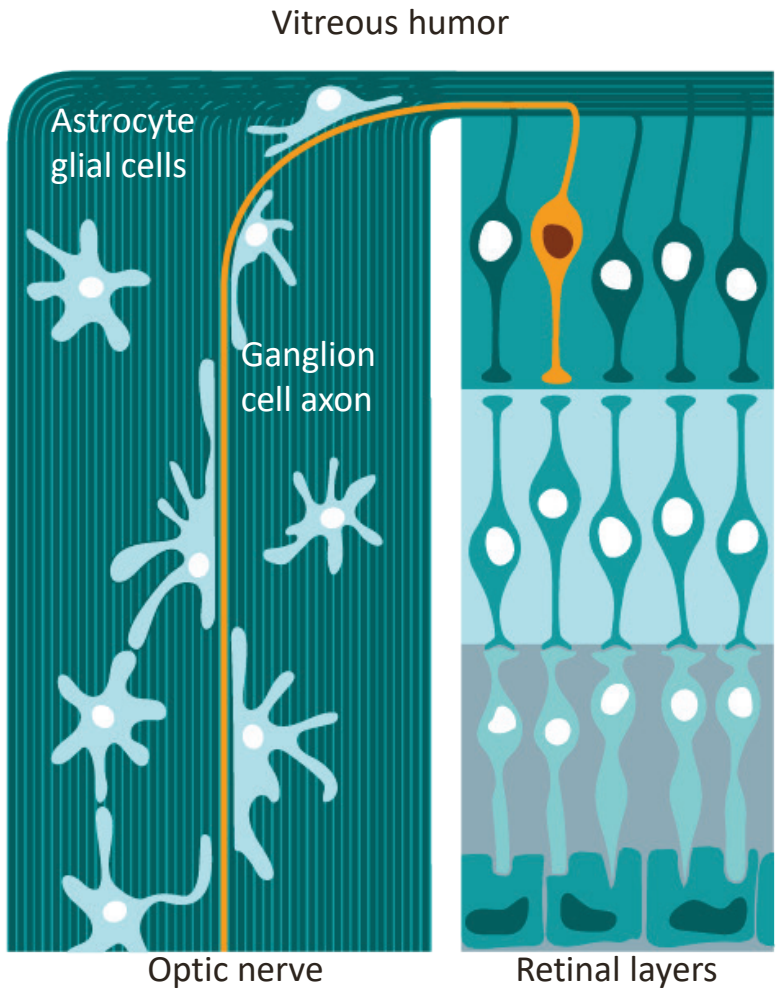
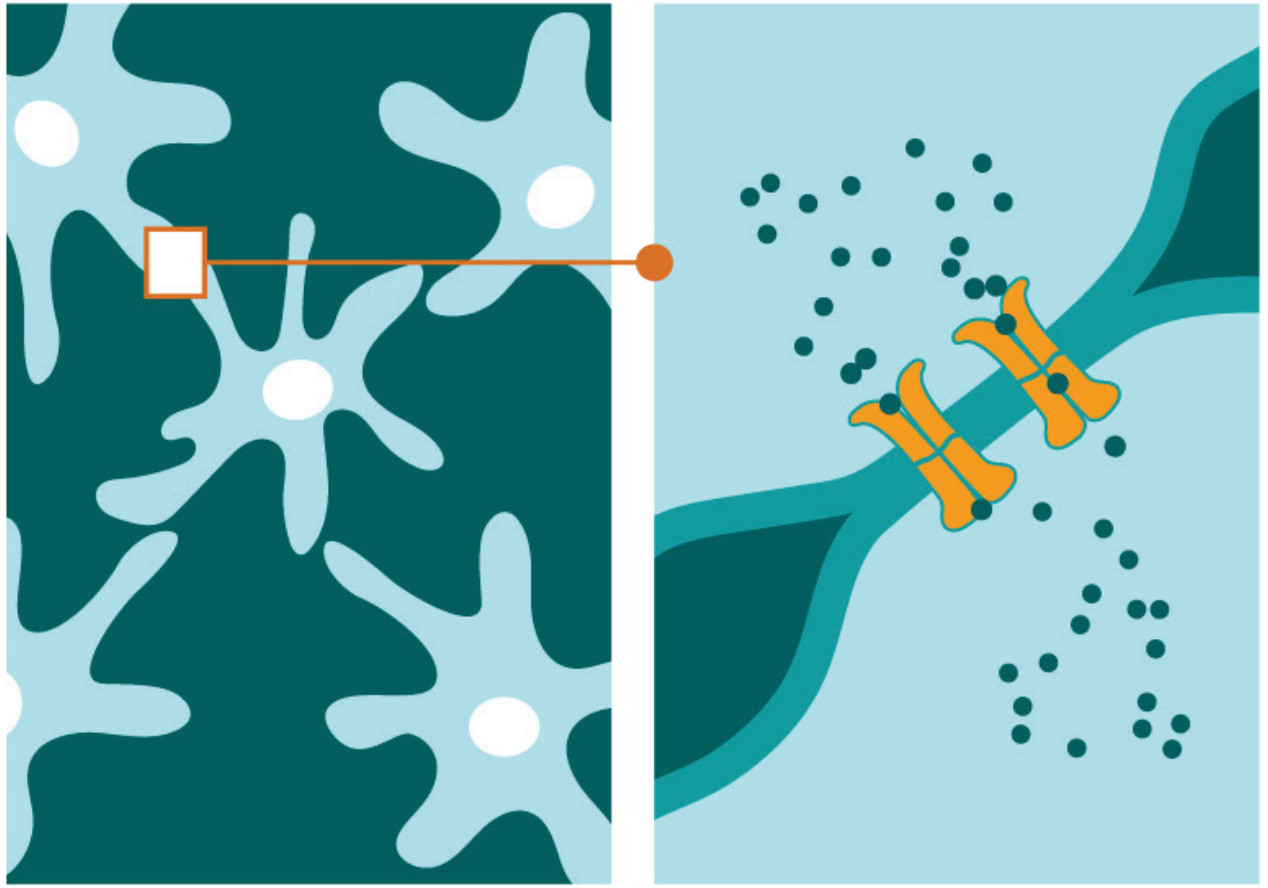
The astrocyte network



Astrocytes in the optic nerve and in the retina form a dense network of interconnected processes.

These connections are mediated by gap junctions, allowing astrocytes to communicate with one another across great distances.



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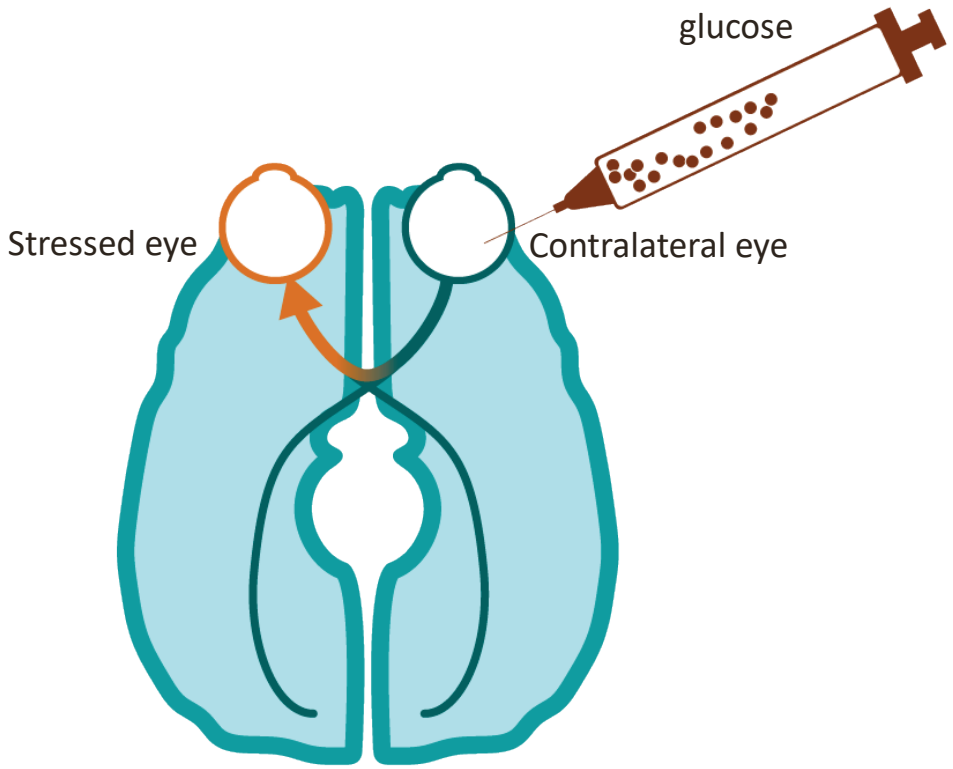
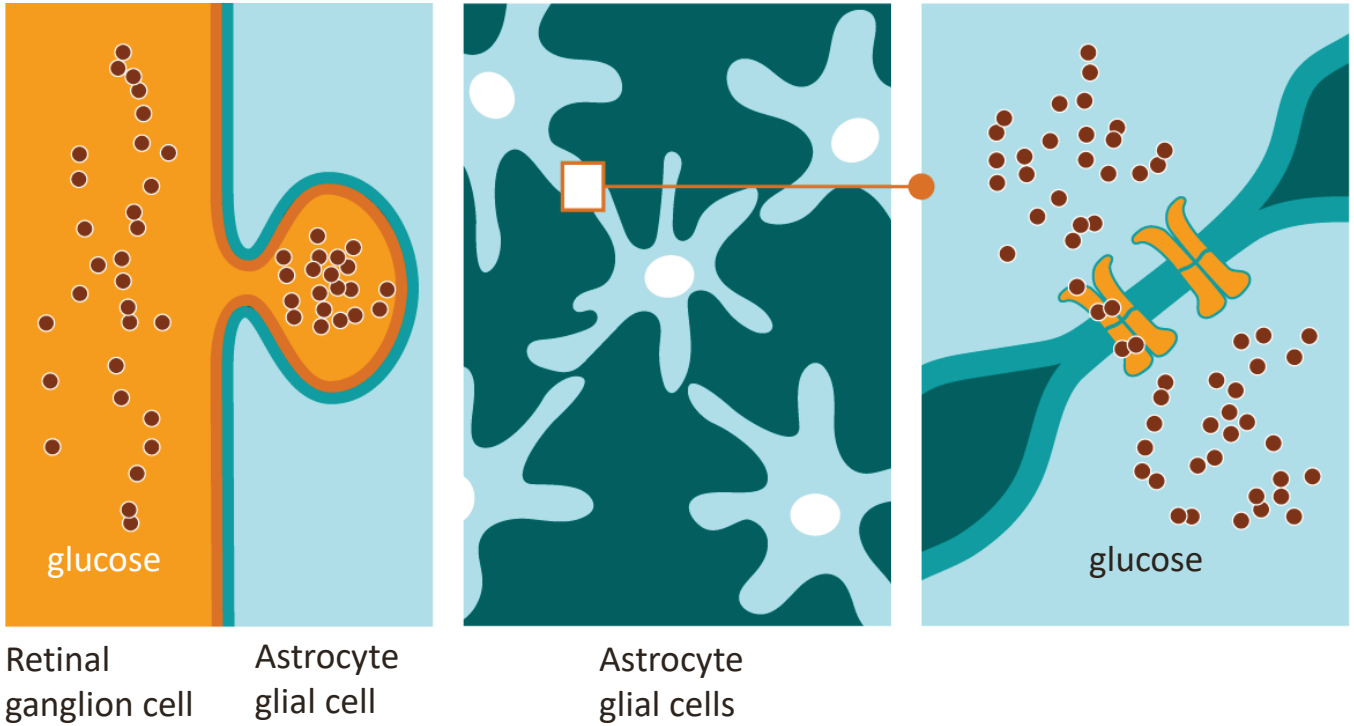
Dr. Calkins' findings



Evidence that in mice under stress conditions, metabolites like glucose transfer from one eye to the other, via astrocytes and specifically their GAP junctions.



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Gensight hypothesis

In humans, other material like mitochondrial metabolites, or even whole mitochondria, could be transferred to the contralateral (untreated) eye via similar mechanisms.