



Corporate Presentation

May 2021

A LEADING Gene Therapy BIOTECHNOLOGY COMPANY

GENSIGHT-BIOLOGICS.COM

Disclaimer

This document contains forward-looking statements and estimates made by the GenSight Biologics S.A. (the “Company”), including with respect to the anticipated future performance of the Company, its subsidiaries and affiliates, and the market in which they operate. They include all matters that are not historical facts. These forward-looking statements can be identified by the use of forward-looking terminology including the terms “developments,” “estimates,” “expects,” “intends,” “may,” “milestones,” “potential,” “value,” “time to market,” “targeting,” “on track,” “planned,” “will,” “move to,” or other variations or comparable terminology, or by discussions of strategy and funding, as well as the Company’s, its subsidiaries’ and affiliates’ technology, and are based on financial and non-financial information, including projections as to the future regulatory situation and other information and assumptions. Such statements, forecasts and estimates are based on various assumptions and assessments of known and unknown risks, uncertainties and other factors, which were deemed reasonable when made but may or may not prove to be correct. Actual events are difficult to predict and may

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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
- Differentiated gene therapy approach forming a technology platform leveraging disruptive gene therapies in ophthalmology and broader
 - Lead product (LUMEVOQ) targets mitochondrial disease
 - Second compound (GS030) uses optogenetic technology

LUMEVOQ® – Filed for Approval in Europe in September 2020 and preparing for commercial launch in early 2022

- **Market:** High unmet medical need; 1,200 – 1,500 new patients / yr EU + US
- **Efficacy:** Unparalleled clinical benefit demonstrated in two Phase III studies
 - +28/+26 ETDRS letters (i.e. over **5 lines** on visual scale) improvement vs nadir(1)
- **Durability & Safety:** Excellent tolerability; Visual improvement maintained at least 3 years post-treatment
 - Clinically meaningful improvement on all Quality of Life parameters at week 96
- **Disease modifying:** Stark difference from Natural History

Commercial strategy and manufacturing capabilities close to completion

- Bilateral injection priced at €700,000 / patient in French named patient Temporary Authorization for Use

(1) Nadir: worst visual acuity from baseline

Established in 2012 / IPO in 2016

EuroNext Paris: SIGHT

Market Cap (May 3, 2021): € 423m

Avg 30-day Daily volume: 1.0% of O/S

Cash (March 31, 2021): € 61.1m

Improvement vs nadir in
REVERSE and RESCUE



Seasoned Executive Team



Bernard Gilly
Chief Executive Officer

PIXIUM VISION (Since 2011)
FOVEA PHARMA (2005-2009)
SOFINNOVA PARTNERS (2000-2005)
TRANSGENE (1992-2000)

Ph.D. in biology and bio-economics



Thomas Gidoïn
Chief Financial Officer

DBV TECHNOLOGIES (2012-2015)
IPSEN (2008-2011)
ERNST & YOUNG (2007-2008)



Magali Taiel
Chief Medical Officer

ProQR THERAPEUTICS (2016-2018)
ELI LILLY (2004-2016)
PFIZER (2001-2004)
SERVIER (1999-2001)

M.D., Board-certified ophthalmologist



Leigh Shaw
VP of Regulatory Affairs

UNITED NEUROSCIENCE (2017-2020)
NIGHTSTARX (2015-2017)
GREGORY FRYER ASSOCIATES (2005-2015)
HUNTINGDON LIFE SCIENCES (2002-2005)
CANTAB PHARMACEUTICALS (1995-2001)



Catherine Cancian
VP of Pharmaceutical Operations

GENETHON (2015-2017)
SANOPI PASTEUR (1998-2014)



Julio Benedicto
VP of Marketing

IMS CONSULTING (2011-2017)
BOOZ & COMPANY (2010-2011)
MONITOR GROUP (1994-2009)



Marie-Claude Holtz
VP of Quality

EXELTIS SANTE (2016-2019)
PFIZER (2015-2016)
ABBVIE (2014-2015)
GALDERMA (2012-2013)
LABORATOIRE LAFON (TEVA) (1993-2012)

Pharm.D.



Isabelle Scarabin
Director, Business Development

LYONBIPOLE (2006-2013)
GREATER LYON (2002-2006)
RESSOURCES EN INNOVATION (1999-2002)
SANOPI PASTEUR MSD (1998-1999)

Pipeline: solid and advanced product portfolio in ophthalmic Gene Therapy

Technology	Product Candidate	Indication	Research	Preclinical	Phase I/II	Phase III	Registration	Expected Approval	
MTS platform	LUMEVOQ® (FDA & EMA Orphan Drug Designation)	LHON ND4 (EU)	[Progress bar spanning Research, Preclinical, Phase I/II, Phase III, and Registration]					Q4 2021	REVERSE: Phase III top-line data reported in Apr (48w) & Oct (72w) 2018 and in May 2019 (96w)
		LHON ND4 (US)	[Progress bar spanning Research, Preclinical, Phase I/II, and Phase III]					Q4 2021	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 Q2 2021
	GS011	LHON ND1	[Progress bar spanning Research and Preclinical]					Initiate preclinical studies following GS010 Phase III clinical data	
	Undisclosed Mitochondrial Target	Undisclosed	[Progress bar spanning Research and Preclinical]						
Optogenetics	GS030 (FDA & EMA Orphan Drug Designation)	Retinitis Pigmentosa (RP)	[Progress bar spanning Research, Preclinical, and Phase I/II]					PIONEER: 3 rd cohort ongoing in PIONEER Phase I/II clinical trial. Report interim data one year after last subject treated	
	GS030	Dry AMD & Geographic Atrophy	[Progress bar spanning Research and Preclinical]						

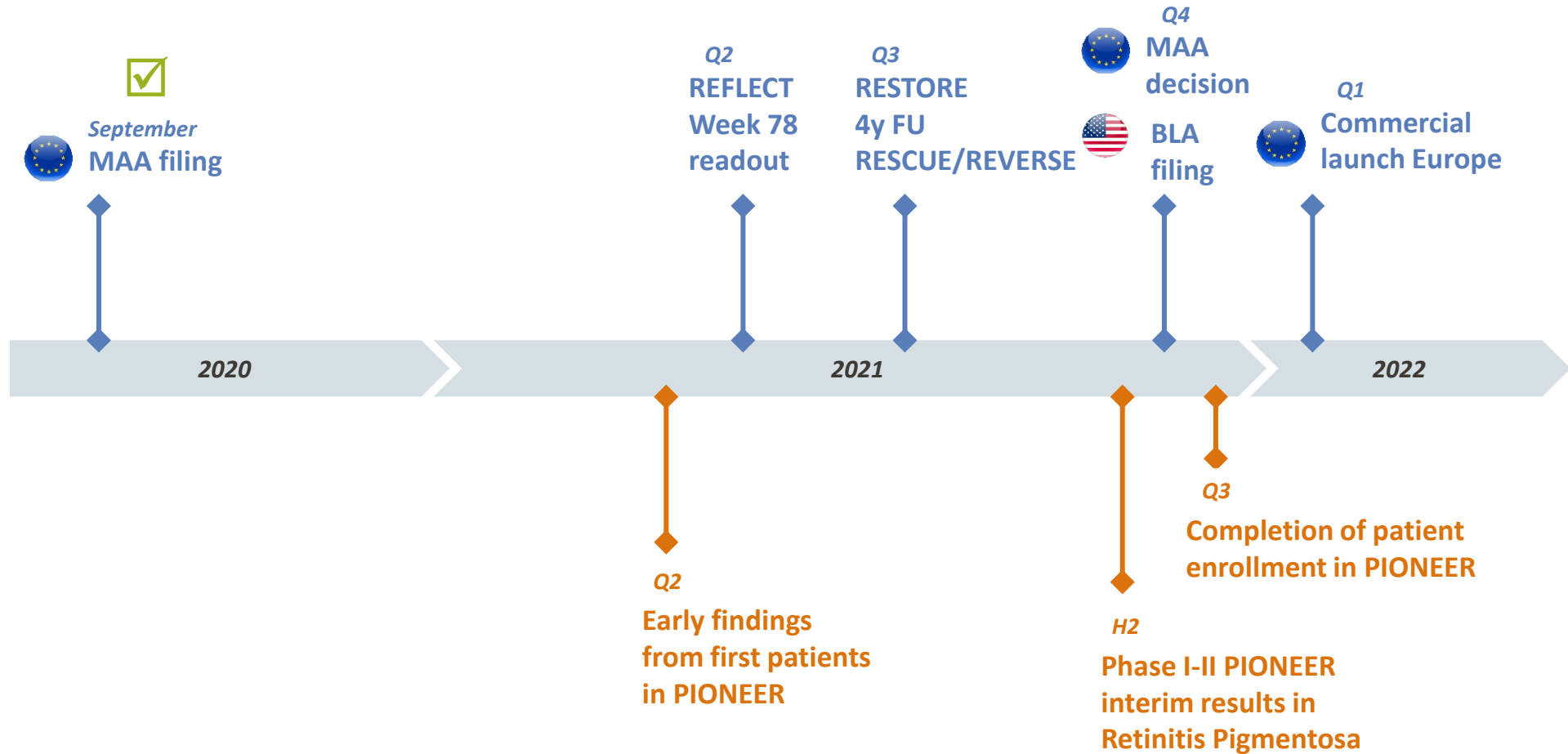
*Conducting this trial under a special protocol assessment with the FDA

Lead candidate, LUMEVOQ® filed for MAA in Europe in September 2020

Rich upcoming news flow with numerous inflexion points



GS030



LUMEVOQ® (GS010) in LHON-ND4

Last Phase III ongoing in Leber Hereditary Optic Neuropathy

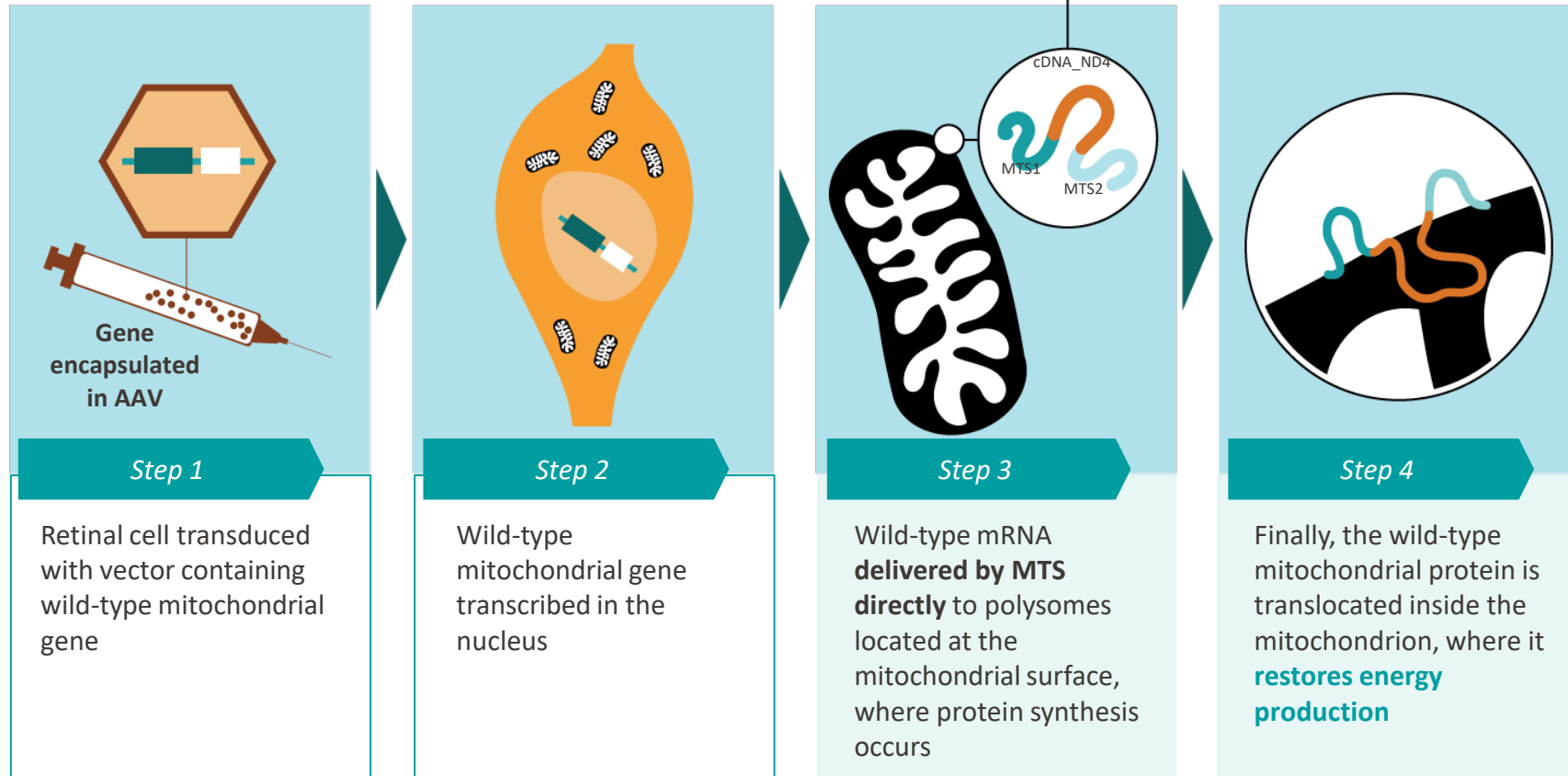
Commercial preparation ongoing for 2022 European launch

A stylized, light teal graphic of a DNA double helix is positioned at the bottom of the slide, extending across the width of the page.

LUMEVOQ® introduces Gene Therapy solution

Replacing affected mitochondrial mRNA via proprietary *MTS* technology*

MTS in action for GS010:

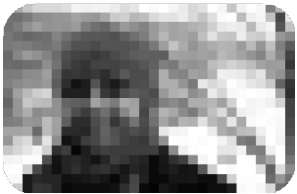


*MTS = mitochondrial targeting sequence

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

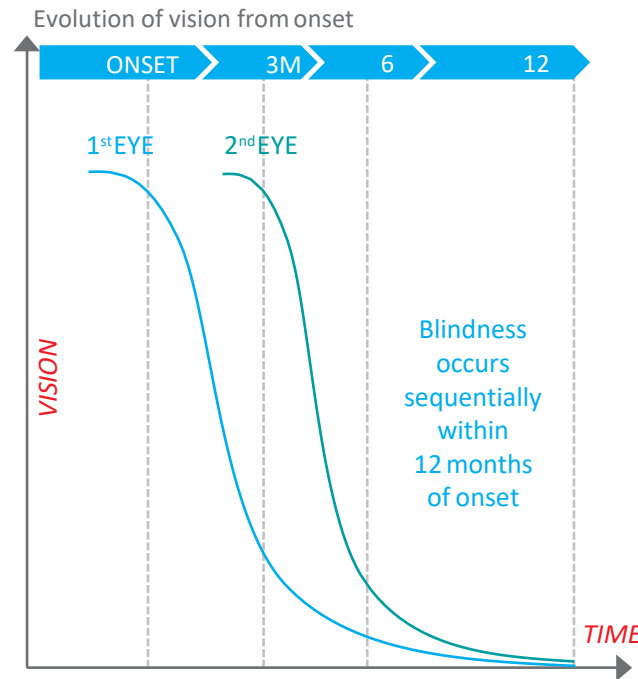


Image source: illustrated from Newman NJ et al., Am J Ophthalmol. 141(6), 1061-1067,2006

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

Unparalleled clinical benefit demonstrated with LUMEVOQ® (GS010) in LHON in two Phase III studies

5 lines bilateral improvement of visual acuity



Change from NADIR in ETDRS letter equivalents		
	Week 96	
	n	Mean (SD)
All-GS010 eyes	37	+28.3 (22.5)
All-sham eyes	37	+24.5 (24.0)



Change from NADIR in ETDRS letter equivalents		
	Week 96	
	n	Mean (SD)
All-GS010 eyes	34	+26.3 (23.9)
All-sham eyes	34	+22.8 (24.2)

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

71% of RESCUE subjects achieved at least 15 letters improvement vs nadir in one or two eyes

- **+28/+26 ETDRS letters (i.e. over 5 lines on visual scale) bilateral improvement vs nadir**
- **Stark difference from natural history outcome**
- **70+% of patients are gaining 15 letters or more**
- **Effect is maintained at least 3 years post administration**
- **Favorable safety profile**

*NADIR is defined as the **worst** BCVA from baseline to Week 96
Mean change from nadir was calculated using observed values (no data imputation)*

Other data complement the finding on sustained bilateral improvement



Insights on Mechanism of Bilateral Effect

- Non-human primate study detected/quantified GS010 viral vector DNA in many tissue samples from contralateral (uninjected) eye



Excellent Tolerability

- No serious adverse events in LUMEVOQ[®]-treated eyes, and no discontinuation due to ocular events
- Most frequently seen ocular adverse events in LUMEVOQ[®]-treated eyes were mainly related to the injection procedure
- Main ocular AE : mild intraocular inflammation – responsive to conventional treatment and without sequelae

Indirect comparison as a cornerstone for EMA Filing

External control group needed because of bilateral improvement in RESCUE and REVERSE trials

- Contralateral effect eliminated the **control group** formed by the sham eyes, as defined in the studies' designs
- EMA scientific advice highlighted the importance of performing an indirect comparison of LUMEVOQ® data using an external control group

Treated Group 76 patients / 152 eyes

- All patients in RESCUE, REVERSE and long-term follow-up study CLIN06 (up to the last available observation)
- Sham eyes included in the treated group, in line with the contralateral effect
 - Treated as independent observations equivalent to injected eyes

Untreated Group (External Control) 208 patients / 408 eyes

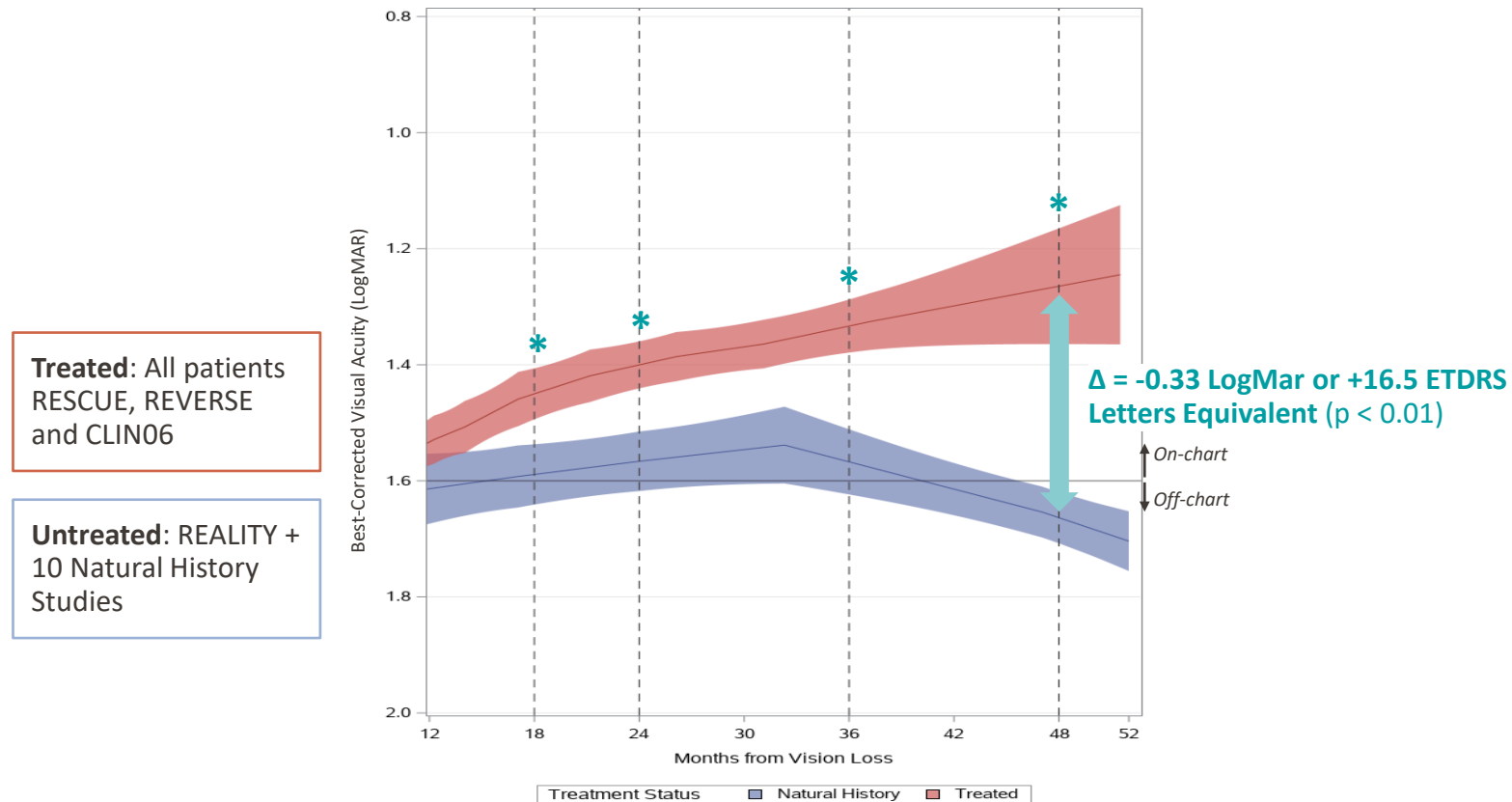
- All patients from REALITY registry study with *ND4* mutation and ≥ 15 years old, *and*
- Patients from 10 natural history studies (2 prospective, 8 retrospective)¹ identified after a systematic review of the LHON scientific literature
 - Must have individual patient data that included mutation type, age, BCVA associated with a time of onset for vision loss
 - Patients included only if they had confirmed *ND4* mutation and were ≥ 15 years old

¹The 10 studies that passed the inclusion criteria were: Hotta 1995, Lam 2014, Nakamura 1993, Newman 1991, Qu 2007, Qu 2009, Romero 2014, Sadun 2004, Yang 2016, and Zhou 2010.

LUMEVOQ[®] modifies disease outcome

Sustained improvement after LUMEVOQ[®] injection vs. absence of recovery among untreated patients

Figure 1. Evolution of Visual Acuity in LUMEVOQ[®]-treated Patients (N=76) versus Untreated Patients (N=208)



Note: All patients had a confirmed G11778A mutation in the *ND4* mitochondrial gene and were at least 15 years old. The diagram shows the Locally Estimated Scatterplot Smoothing (LOESS) curves for visual acuity in LUMEVOQ[®]-treated patients and untreated patients. The shaded areas represent the 95% confidence interval for the mean BCVA. "Treated" eyes refer to all eyes (LUMEVOQ[®] and sham) from the RESCUE, REVERSE and CLIN06 trials (N=76 patients / 152 eyes). Untreated eyes refer to patient-level data from the REALITY study and a matched data set from two prospective and eight retrospective natural history studies¹ (N=208 patients / 408 eyes).

*Statistically significant difference between mean visual acuity of treated and untreated eyes at M18, M24, M36 and M48, as illustrated by the non-overlapping confidence intervals.

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



————— Considered clinically relevant difference*

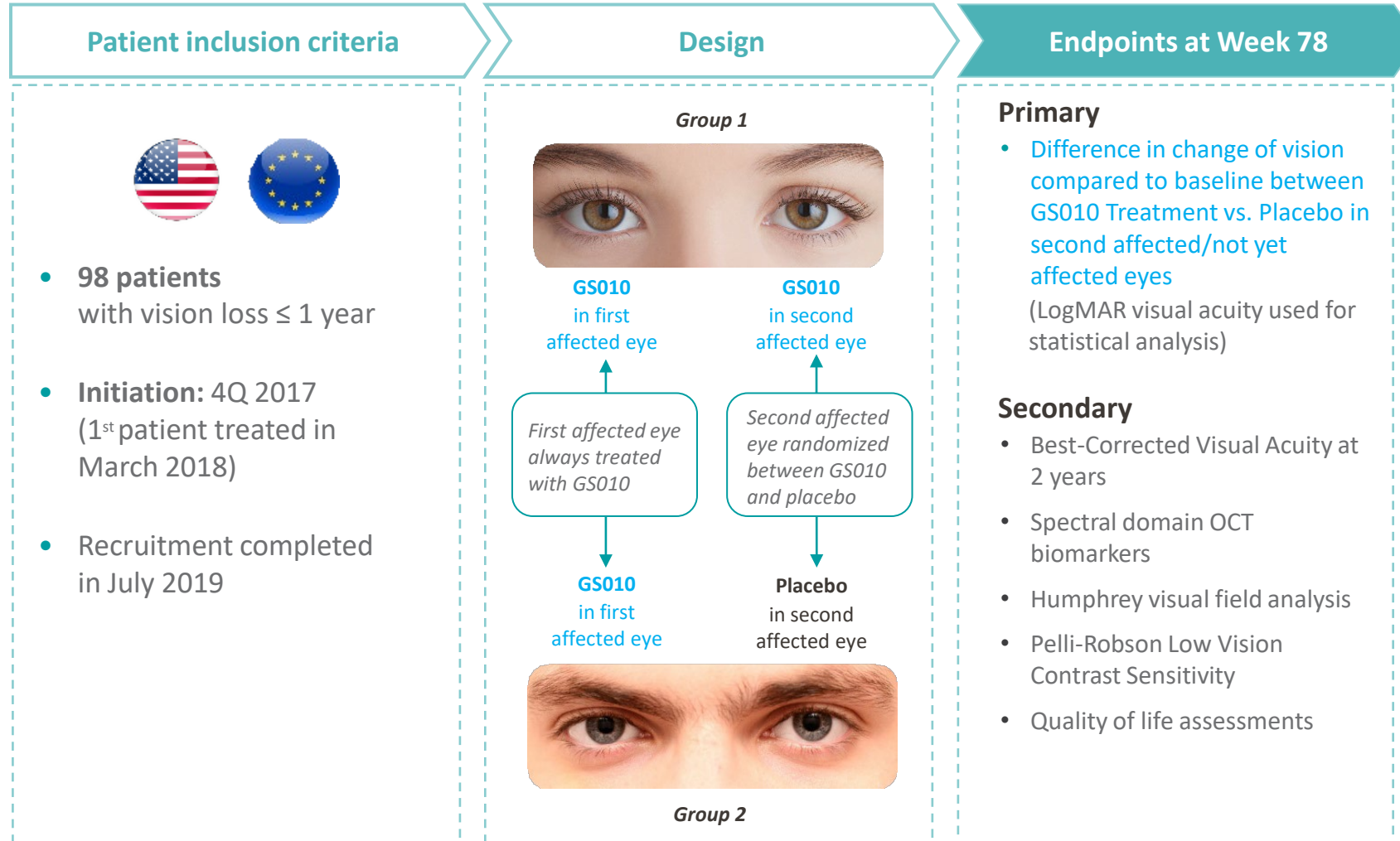
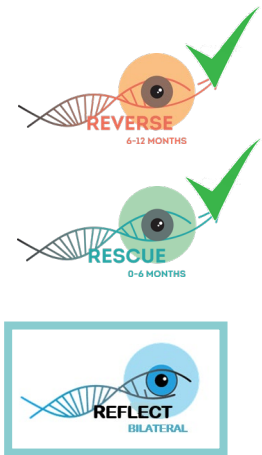
* Suñer *et al.* (2009): clinically relevant score differences based on a clinically significant 15-letter BCVA improvement at 12 months.

** The composite score is an average of the vision-targeted sub-scale scores, excluding the general health rating question.

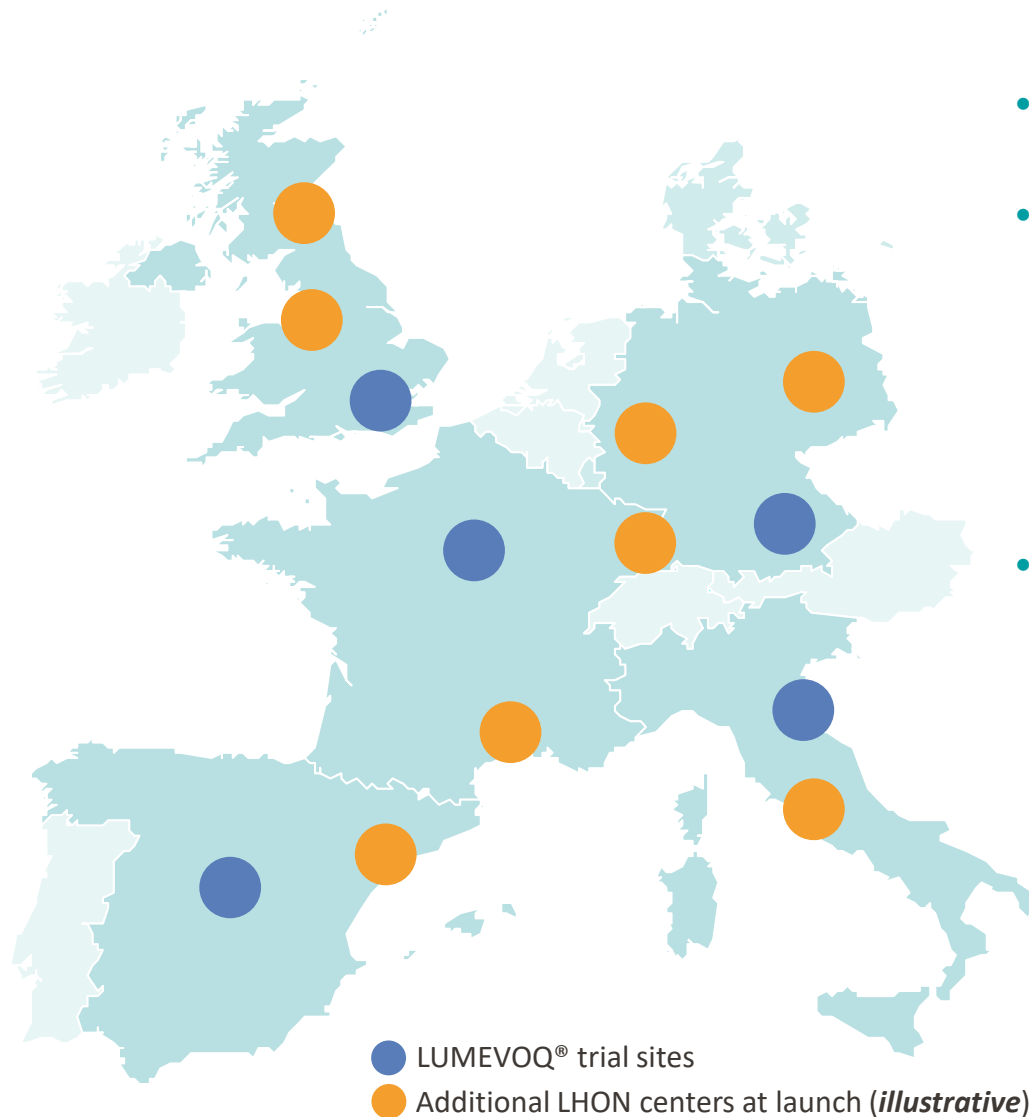
Last ongoing Phase III trial: REFLECT to assess efficacy and safety of bilateral injection

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

Q2 2021
LUMEVOQ®
REFLECT
Week 78
Read-out

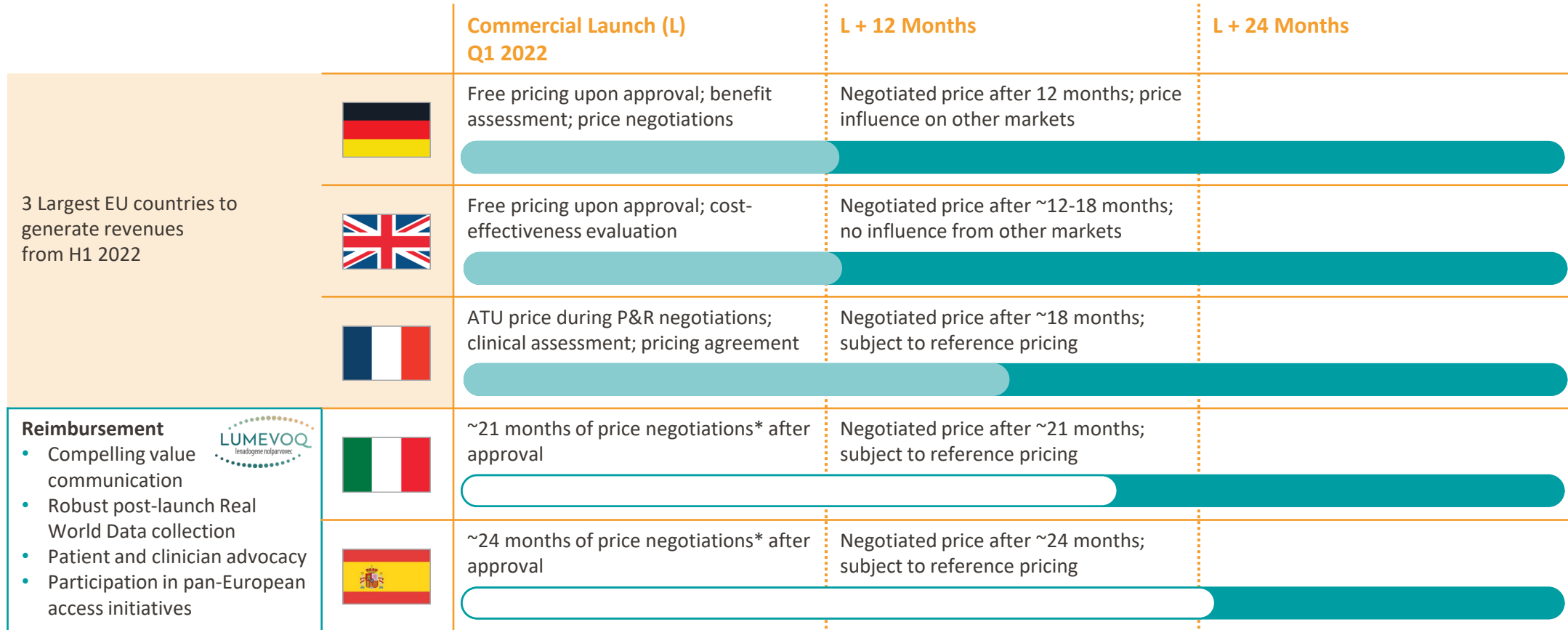


European Commercial Strategy – Leveraging LUMEVOQ® Trial Centers to Build Network of LHON Centers of Excellence



- LHON experts mapped in both major and smaller markets
- Progressively build the LHON clinical network working with LHON experts
 - Recognize varying levels of LHON expertise and patient mobilization across markets
 - Balance patient reach with logistical complexity
- LHON expert- and LHON patient-centric commercial and medical teams executing focused local activities
 - Foster existing relationship with centers and LHON experts
 - Broaden LHON expert network locally and internationally
 - Manage patient and caregiver experience along the patient journey

European Reimbursement Strategy – Short Term Revenues Generation Expected in H1 2022



Note: Duration of negotiations depicted is based on industry benchmarks for recent rare disease launches; timings are illustrative

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



- 8 individual patients Expanded Access INDs so far approved by the FDA for LUMEVOQ® (lenadogene nolparvovec)
- These 8 subjects have been treated (bilateral GS010 IVT) under the investigator-sponsored programs since 2019
- Additional 10 individual patients Expanded Access INDs to be processed



ansm

- “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
 - €4.4M revenues generated in 2020
 - Increasing demand from physicians in 2021
 - Reimbursement warranted by the national Social Security up to €30M/year
- Named-Patient or Cohort Expanded Access Programs (EAP) in other European countries being set up to leverage LUMEVOQ® treatment for the benefit of patients accross Europe and beyond

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



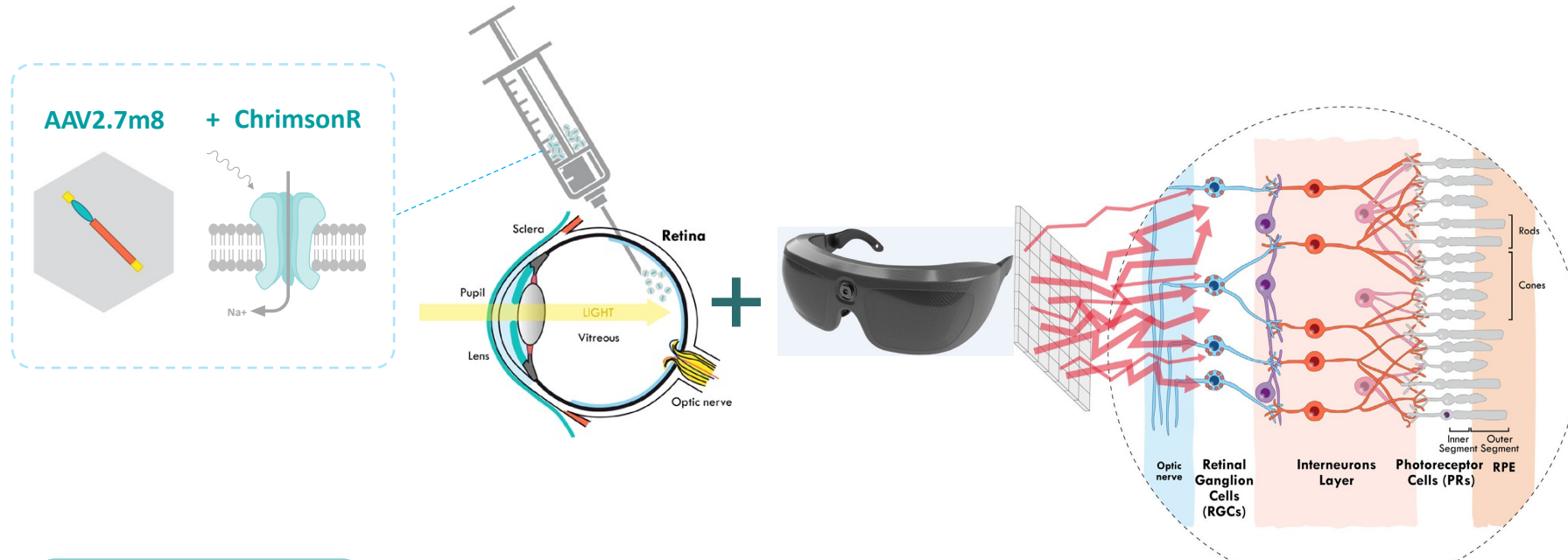
- Blinding genetic disease caused by mutations in over 100 different genes
- Sequential photoreceptor degeneration leads to slow & irreversible progression to blindness, usually at age 40-45
- 15-20,000 new patients each year in the US and EU

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

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



The product of research collaboration with



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

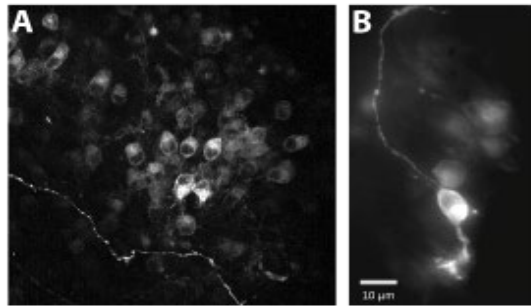
Retinal output sent to brain for image processing

GS030 leads to functional vision restoration in monkey and rats

Localization of light-sensitive protein in NHP retina

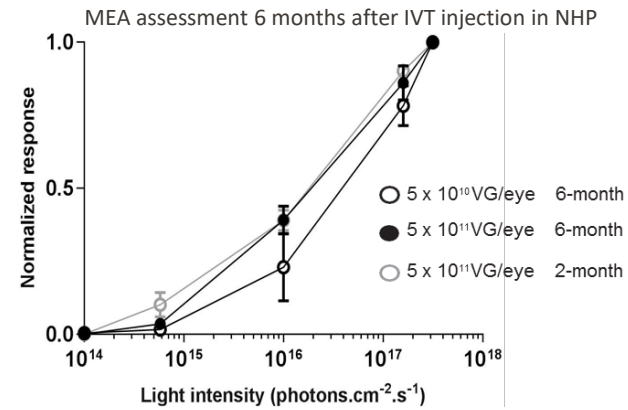
Expression of ChrR-tdT in midget cells of monkey perfovea

In vivo in NHP assessment 6 months after IVT injection



Dose-ranging response to firing relationship in NHP

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



Recent publication

Optogenetic therapy: high spatiotemporal resolution and pattern discrimination compatible with vision restoration in non-human primates. Gauvain G. et al.

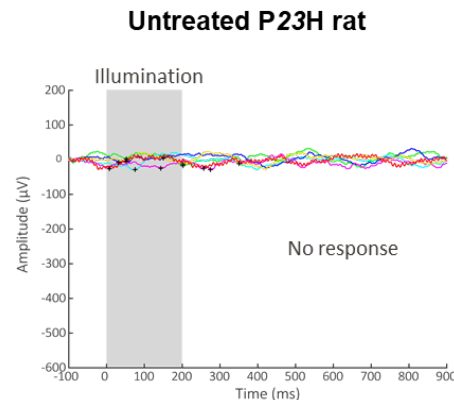
Communications Biology, Feb. 2021

<https://www.nature.com/articles/s42003-020-01594-w>

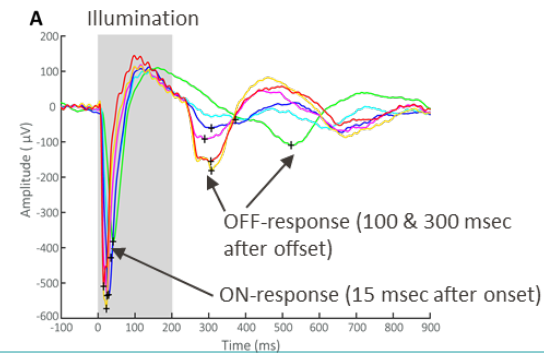
Restoration of a functional vision in P23H rats

Light-induced visual evoked cortical responses

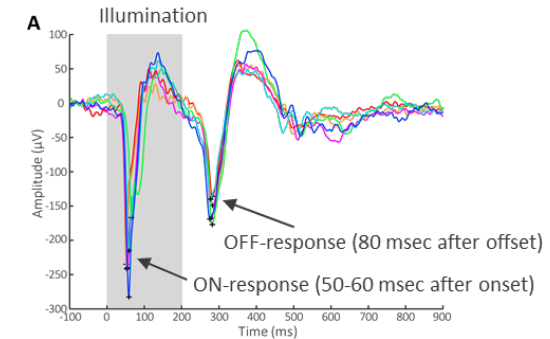
Full field 590 nm light from $\sim 4.7 \times 10^{15}$ to 1.1×10^{17} photons/cm²/sec



GS030-treated P23H rat



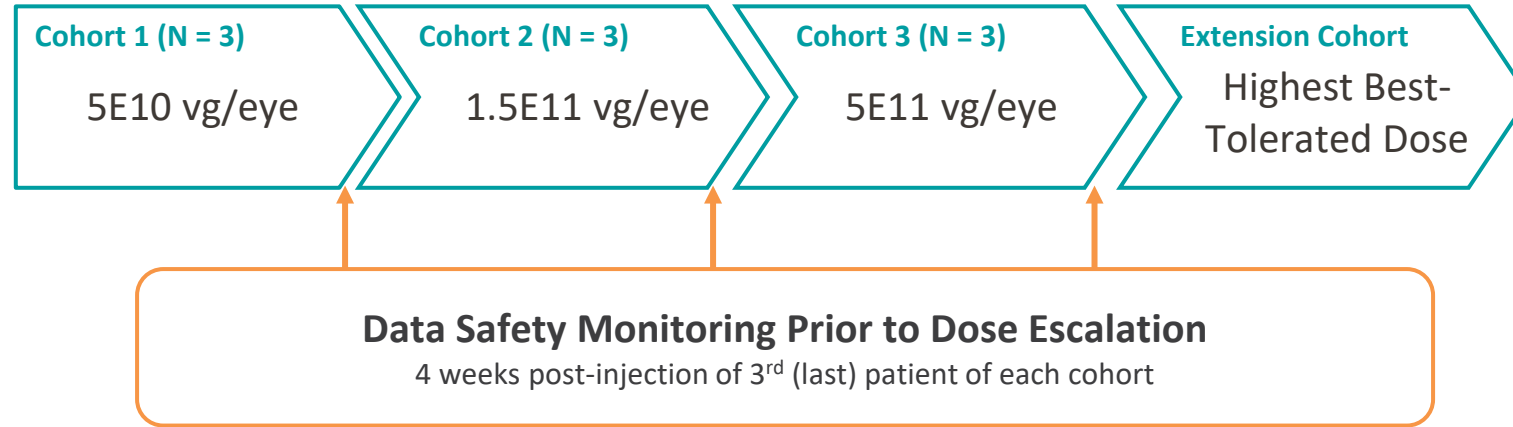
Normal Long-Evans rat



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



Study design



- **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

Cohort 3 ongoing without any modification after DSMB#2 approval

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

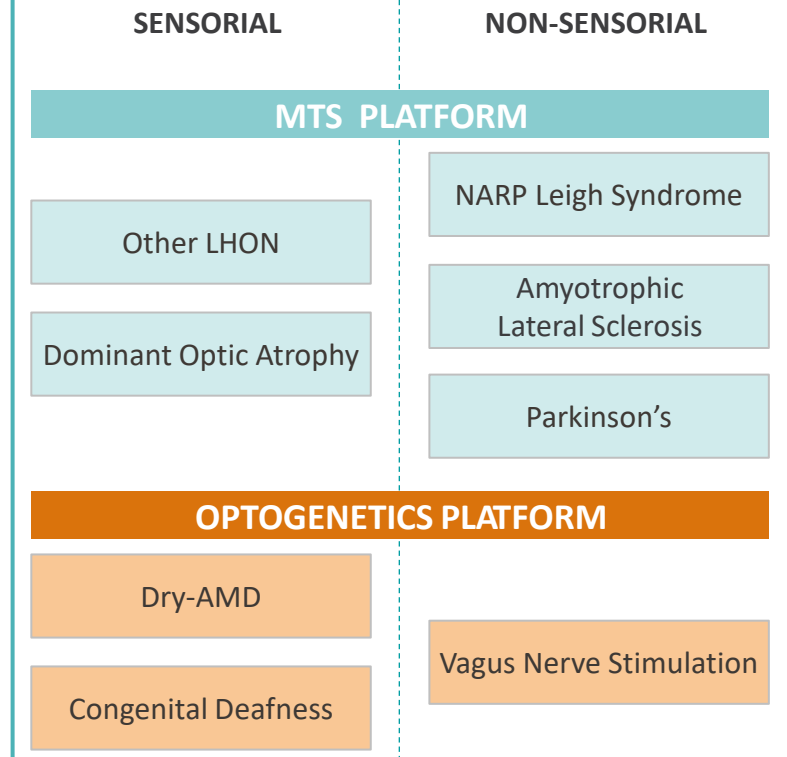
GenSight at the forefront of Gene Therapy with potential product launch in 2021

- » **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 targeting large number of sensorial and non-sensorial diseases



GenSight Biologics in numbers

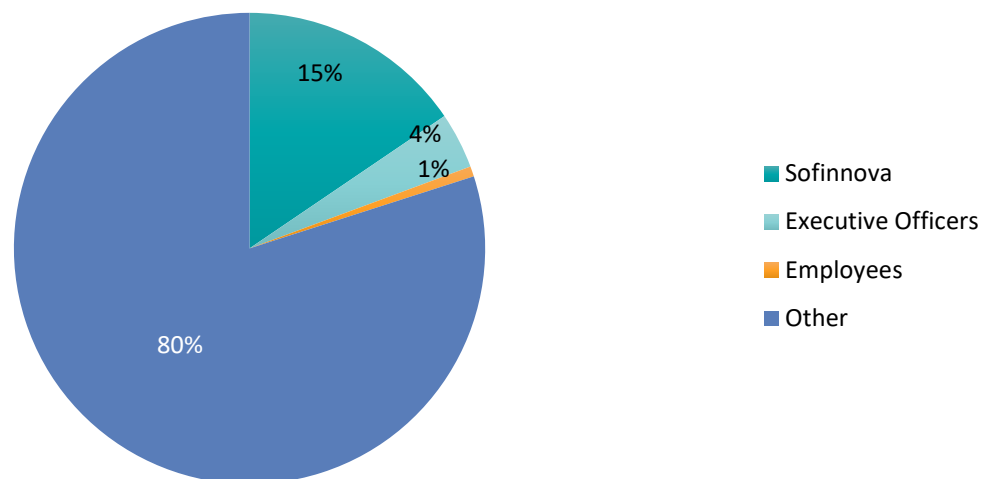
Key financial information

Company Overview

Market Cap*:	€ 423m	Analyst Coverage
Cash Position (March 31, 2021):	€ 61.1m	• Chardan: Gbola Amusa (US)
Outstanding Shares:	46.0m	• Bryan Garnier: Dylan van Haaften (FR)
Latest Amount Raised (March 2021):	€ 30m	• Oddo BHF: Sébastien Malafosse (FR)
Raised to date	€ 197m	
IPO Date	July 2016	

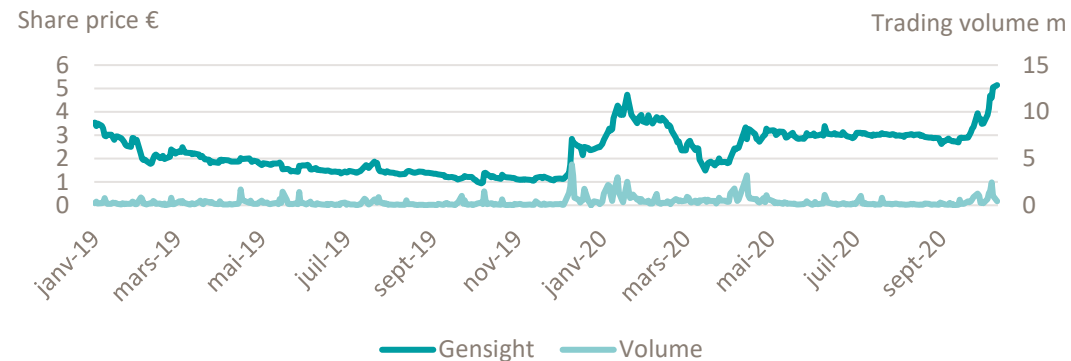
*As of May 3, 2021

Shareholder structure



As of May 2021

Share price evolution and trading volume



Corporate calendar

Date

2020 Full-Year Financial Update and Statements	March 10, 2021
2021 1Q Cash Position	April 20, 2021
Annual General Meeting	April 29, 2021
2021 First-Half Financial Update and Statements	July 29, 2021
2021 3Q Cash Position	October 19, 2021
2021 4Q Cash Position	January 18, 2022

Appendix



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

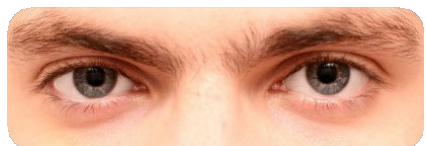
Group 1



GS010
in right eye

SHAM
in left eye

Group 2



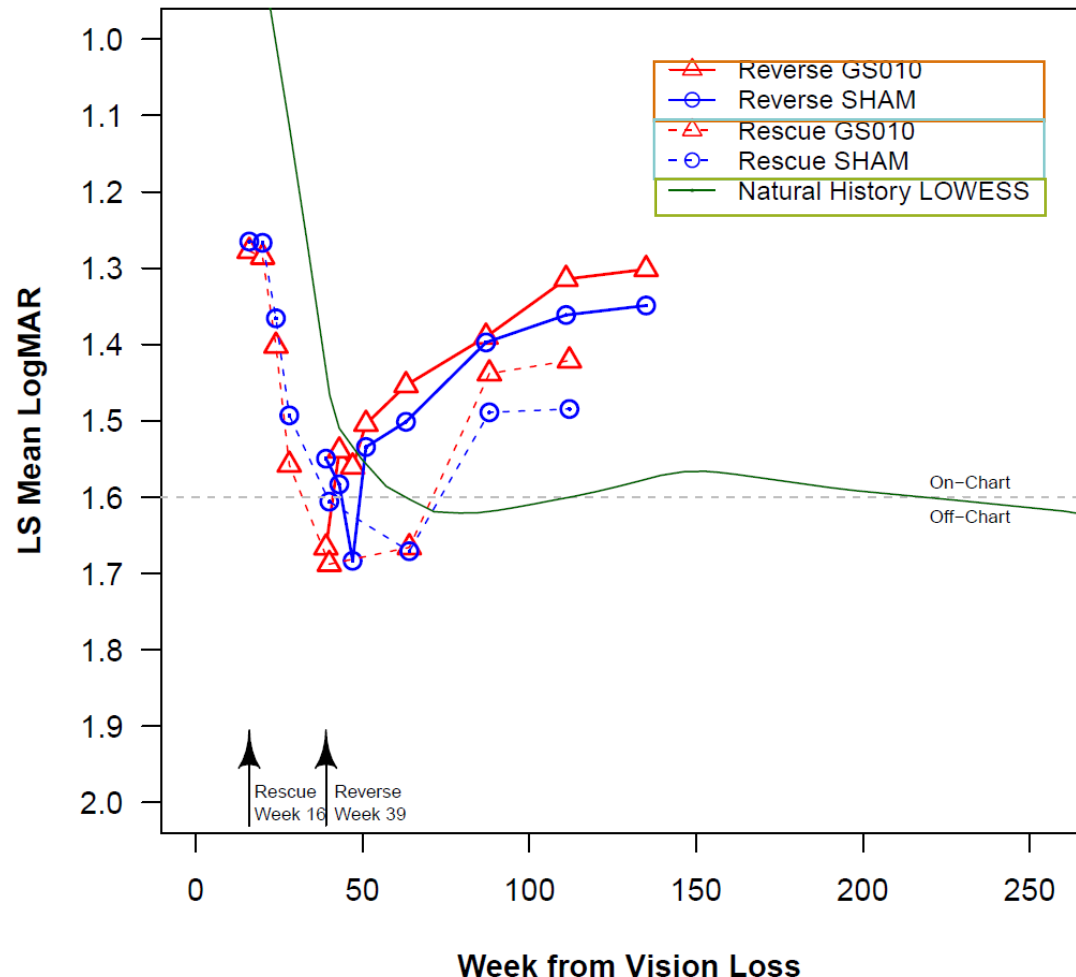
SHAM
in right eye

GS010
in left eye

RESCUE



- Onset of disease **≤ 6 months**
- 39 patients enrolled



+28 ETDRS Letters vs nadir



+26 ETDRS Letters vs nadir

REVERSE and RESCUE: Final Results
75 ND4 Subjects ≥ 15 years old – Over 2 year-follow-up



Retrospective Natural History

REALITY: Final Results
23* ND4 Subjects ≥ 15 years old – Over 5 year-follow-up

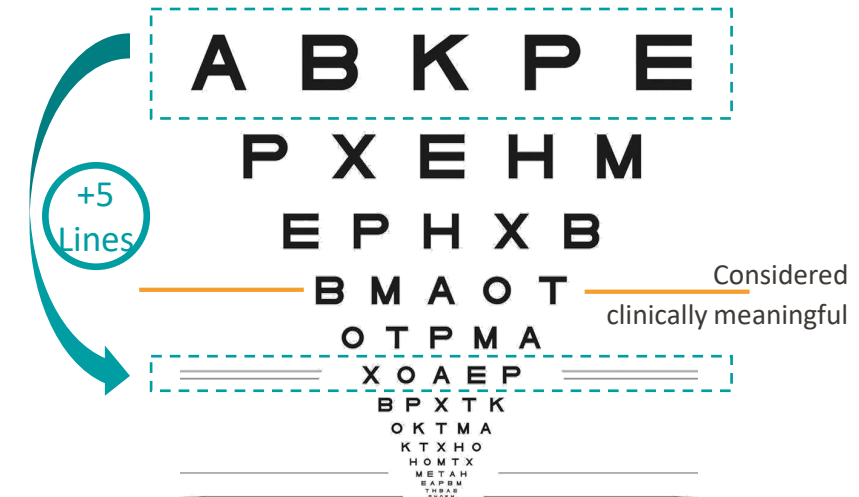
*: Out of which, 15 had been treated with idebenone, the majority within 12 months of their vision loss

Visual Acuity: Improvement of BCVA from NADIR

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



Change from NADIR in ETDRS letter equivalents			Change from NADIR in ETDRS letter equivalents		
Week 96			Week 96		
	n	Mean (SD)		n	Mean (SD)
All-GS010 eyes	37	+28.3 (22.5)	All-GS010 eyes	34	+26.3 (23.9)
All-sham eyes	37	+24.5 (24.0)	All-sham eyes	34	+22.8 (24.2)



NADIR was defined as the **worst BCVA** from baseline to Week 96
 Mean change from nadir was calculated using observed values (no data imputation)

**Unparalleled clinical benefit demonstrated with LUMEVOQ® (GS010) in LHON in two Phase III studies:
 +28/+26 ETDRS letters (i.e. over 5 lines on visual scale) improvement vs nadir**

3-year long-term follow-up: sustained efficacy and safety

Change from NADIR in ETDRS letter equivalents			
		Year 2 post-injection	Year 3 post-injection
	n	Mean (SD)	Mean (SD)
All-GS010 eyes	61	+18.8 (15.3)	+20.5 (18.3)
All-sham eyes	61	+17.3 (14.6)	+19.4 (18.5)

The CLIN06 sample consists of the RESCUE and REVERSE participants who accepted to be followed in the CLIN06 study

NADIR was defined as the **worst BCVA** from baseline to Week 96 and 144
Mean change from nadir was calculated using observed values (no data imputation)



n=37



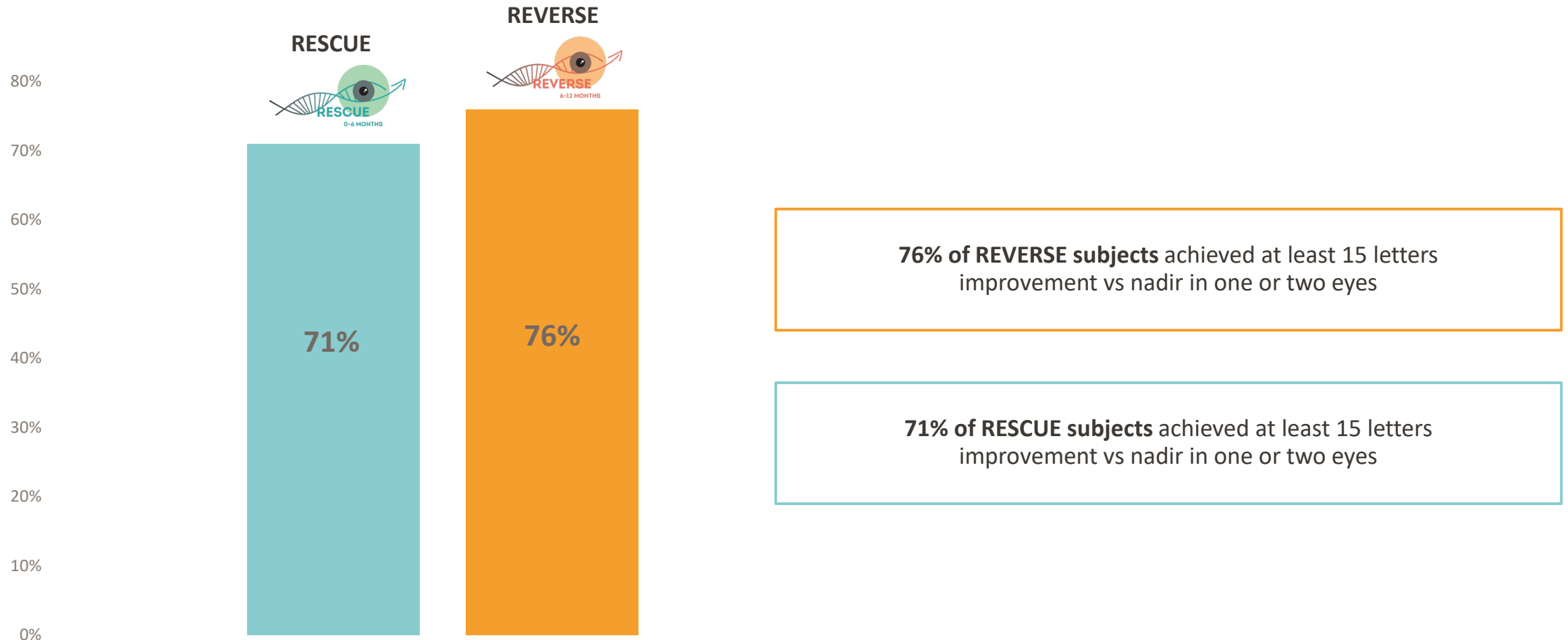
n=34

**CLIN06 – long-term
follow-up study**

n=31

n=30

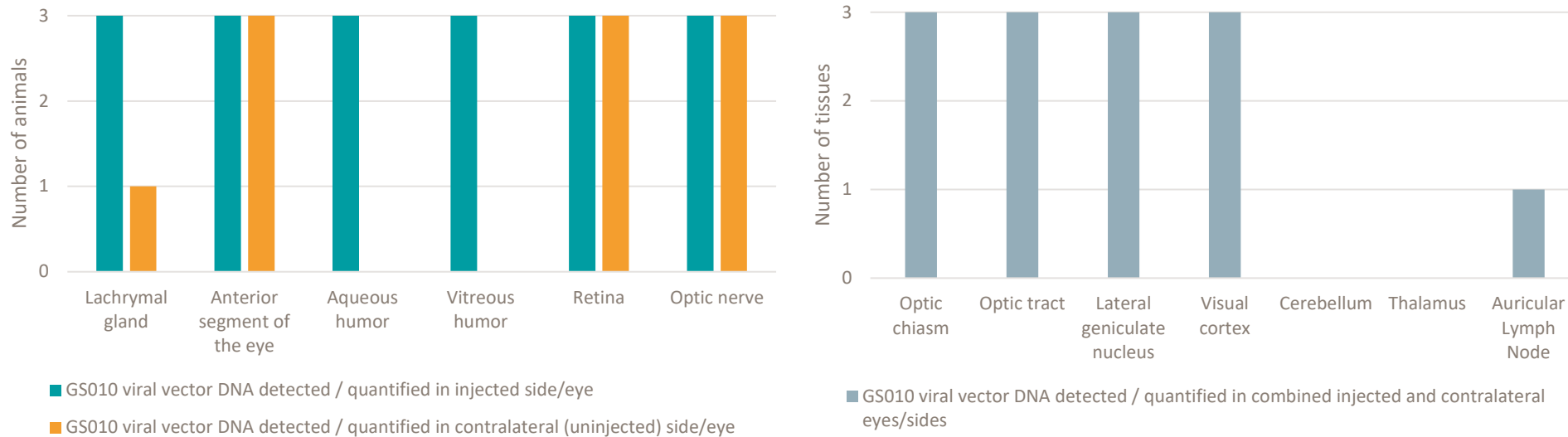
REVERSE and RESCUE demonstrate that over 70% of patients benefit from treatment



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

Presence of GS010 DNA in the visual and cerebral systems of test monkey at 3 months after GS010 injection



- 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 bar graph indicates the number of animals (of three) in which rAAV2/2-ND4 (GS010) DNA was present above the limit of detection (15.6 copies/μg of DNA).

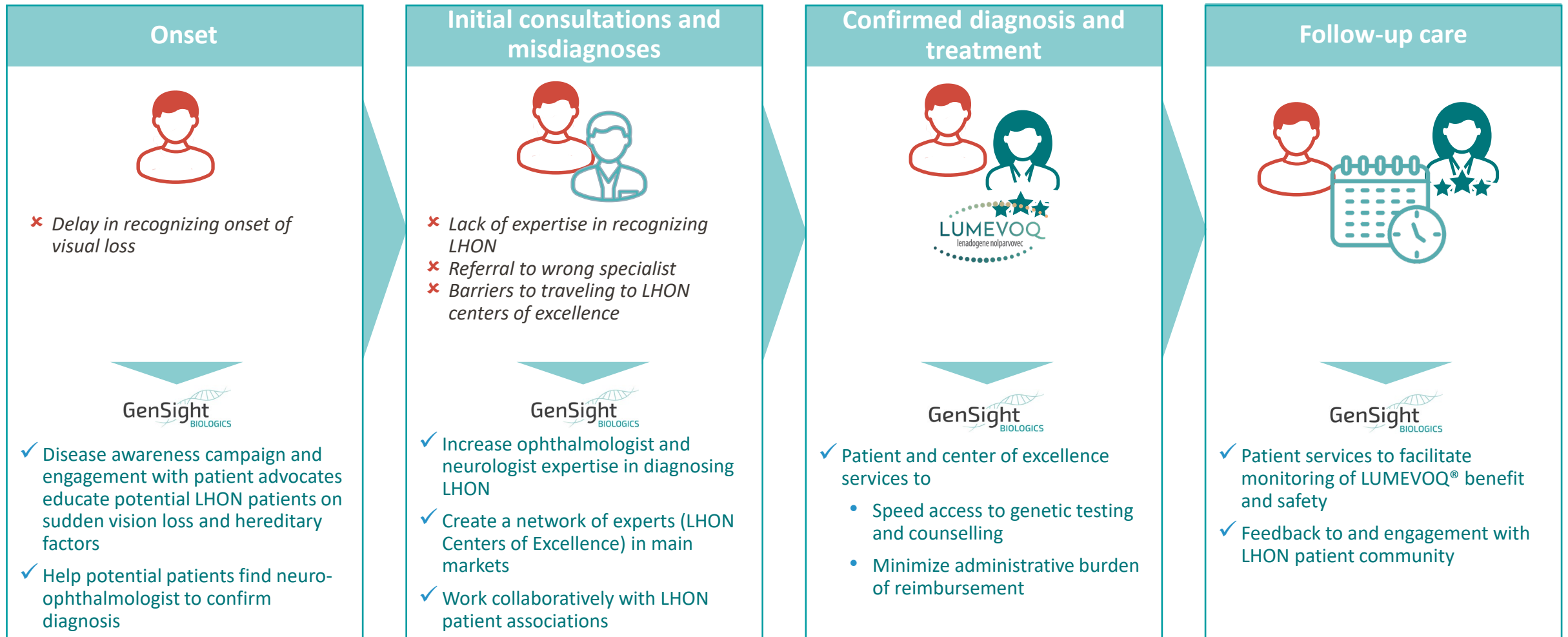
Source: Yu-Wai-Man P. et al., Sci Transl Med. Dec. 2020

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











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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.

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	The optimized allotopic expression of ND1 or ND4 genes restores respiratory chain complex I activity in fibroblasts harboring mutations in these genes	Bonnet C. et al., Biochim Biophys Acta., May 2008
	Optimized allotopic expression of the human mitochondrial ND4 prevents blindness in a rat model of mitochondrial dysfunction	Ellouze S. et al., Am J Hum Genet., Sep. 2008
	Nuclear expression of mitochondrial ND4 leads to the protein assembling in complex I and prevents optic atrophy and visual loss	Cwerman-Thibault H. et al., Mol Ther Methods Clin Dev., Feb. 2015
	Safety of rAAV2/2-ND4 Gene Therapy for Leber Hereditary Optic Neuropathy	Vignal C. et al., Ophthalmology, Feb. 2018
	Immune Response and Intraocular Inflammation in Patients With Leber Hereditary Optic Neuropathy Treated With Intravitreal Injection of Recombinant Adeno-Associated Virus 2 Carrying the ND4 Gene: A Secondary Analysis of a Phase 1/2 Clinical Trial	Bouquet C. et al., JAMA Ophthalmol., April 2019
	Bilateral visual improvement with unilateral gene therapy injection for Leber hereditary optic neuropathy	Yu-Wai-Man P. et al., Sci Transl Med. Dec. 2020
	Efficacy and safety of intravitreal gene therapy for Leber hereditary optic neuropathy treated within 6 months of disease onset	Newman N.J. et al., Ophthalmology, Jan. 2021
		
	Safety of intravitreal gene therapy for treatment of subjects with Leber Hereditary Optic Neuropathy due to mutations in the mitochondrial ND4 gene – The REVEAL study	Vignal-Clermont C. et al. BioDrugs, Feb. 2021
	Natural History of Patients with Leber Hereditary Optic Neuropathy – Results from the REALITY Study	Yu-Wai-Man P. et al., Eye, April 2021