GenSight BIOLOGICS

A LEADING GENE THERAPY BIOTECHNOLOGY COMPANY

Corporate presentation

SEPTEMBER 2024

GENSIGHT-BIOLOGICS.COM

Disclaimer

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GenSight BIOLOGICS

A new chapter, led by a seasoned international management team

Laurence Rodriguez

Chief Executive Officer

SANOFI (2011-2021)
GENZYME (2005-2011)
FRESENIUS (1998- 2005)
NUTRICIA/DANONE (1994-1998)

Magali Taiel

Chief Medical Officer

ProQR THERAPEUTICS (2016-2018)
ELI LILLY (2004-2016)
PFIZER (2001-2004)
SERVIER (1999-2001)
M.D., Board-certified ophthalmologist

Scott Jeffers

Chief Technical Officer

REDPIN THERAPEUTICS (2021-2022)
UNIQURE (2019-2021)
SELECTA BIOSCIENCES (2018-2019)
BRAMMER BIO (2015-2018)
Ph.D. in virology

Magali Gibou

VP, Regulatory & Quality

SANGAMO THERAPEUTICS (2019-2023)
HOFFMANN LA ROCHE (2014-2019)
TRANSGENE (2007-2014)

Jan Eryk Umiastowski

Chief Financial Officer

BLUEBALLOON CAPITAL (2023-2024)
CEGEDIM (2007 -2023)
AMAS BANK (2005-2007)
JET FINANCES (2002-2005)

Julio Benedicto

SVP, Strategy & Operations

IMS/IQVIA (2012-2017)
BOOZ AND COMPANY (2011)
MONITOR DELOITTE (1994-2010)

Marion Ghibaudo

Chief Technical Device Officer

MAUNA KEA TECHNOLOGIES (2018 – 2021)
L'OREAL (2009 – 2018)
Ph. D. in biophysics

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International board of directors with extensive industry and geographic expertise

Michael Wyzga

Chairman since March 2016

Corporate strategy

- Various senior positions at Genzyme Corporation
- Chairman: X4 Pharmaceuticals, Mereo Pharmaceuticals
- Board member: LogicBio, Adagiotherapeutics, Akebia therapeutics
- President of MSW Consulting Inc.

Prof. José-Alain Sahel

Observer and Co-Founder

Research and development

- Founding Chair, Vision Institute, Paris
- Professor and Chair, Dept. of Ophthalmology, Univ. Of Pittsburgh
- Winner, 2024 Wolf Prize in Medicine

Maritza McIntyre, Ph.D.

Independent Director

CMC and Regulatory Affairs

- 20 years of experience in development of biological molecule products in biotech firms and FDA
- Bavarian Nordic, REGENXBIO, Nanocor therapeutics, bamboo therapeutics
- President of Advanced Therapies Partners LLC

Simone Seiter, M.D., Ph.D.

Independent Director

Commercialization and launch excellence

- 30 years of experience in pharmaceutical Industry Simon Kucher and IQVIA
- Execution on global, regional and local level
- Board member: GenSight Biologics, Mediphage

Françoise de Craecker

Independent Director

Commercialization and operational excellence

- 40 years of experience in Pharmaceutical Industry
- Local, Regional and Global responsibilities
- Orphan Drugs in multiple Therapeutic Areas and Gene Therapies

Elsy Boglioli

Independent Director

Biotech scale-up and BD

- 15 years of experience in biotech industry
- Director at Womed, InPart, Metafora, FTI consulting
- Former COO Cellectis, Former Partner and MD at The Boston Consulting Group

Cedric Moreau

Representing Sofinnova Partners

Finance

- 18 years of experience in life sciences investment banking, 10 years of experience as a Healthcare equity analyst
- Managing director at ODDO BHF, Bryan Garnier

William Monteith

Independent Director

Manufacturing

- 43 years of experience in both small molecule and large molecule pharmaceutical manufacturing
- Program Director, North Carolina Life Sciences Biomanufacturing Forum

Investment case
Gene therapy company with a pivotal stage lead product candidate

Late-stage Biotech company

Public company founded in 2012. Publicly listed on Euronext Paris (SIGHT).

Exclusive focus on developing and commercializing **gene therapies** for neurodegenerative retinal diseases and diseases of the central nervous system → **lead product in late-stage clinical development with a targetable market of 800-1,000 patients per year in the US and EU**

Seasoned management team/ Solid investor base

Management team with **strong and highly relevant Biotech experiences** in R&D and commercialization.

Solid investor base of Healthcare specialist investors, including EU and US based investors.

LUMEVOQ® Robust clinical data in LHON

Phase I/II and four Phase III studies in Leber Hereditary Optic Neuropathy (*ND4* LHON) show ability to improve vision in a blinding, acutely progressing and irreversible disease.

No further clinical trial required for submission in the UK; **new Phase III trial to address US and EU** requirements received positive feedback on overall design.

LUMEVOQ® Defining registration pathway

Past **manufacturing** issues successfully remediated.

To be available in France through **paid Early Access** (pending product availability in **October 2024**).

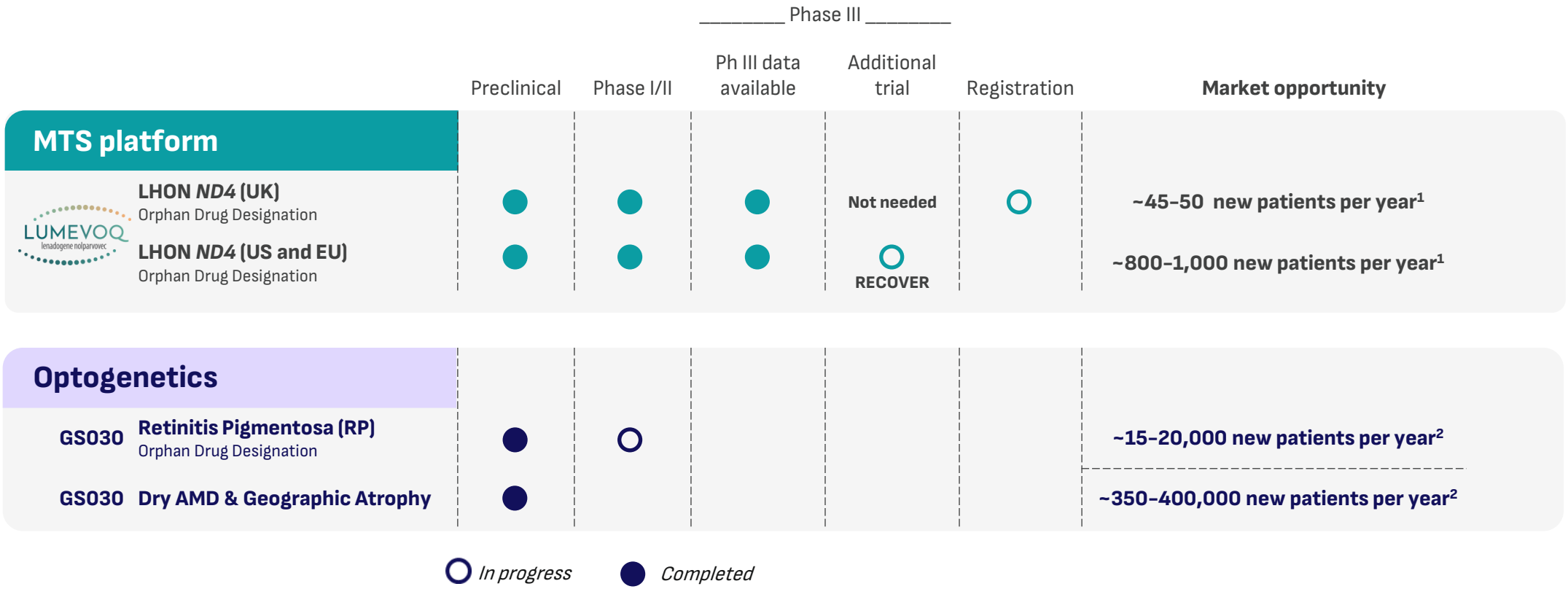
Ongoing discussions with EMA, UK MHRA and US FDA to confirm registration pathway.

Cutting-edge optogenetics in Retinitis Pigmentosa

GS030 outstanding early findings for mutation-agnostic treatment: decades-long blind patients reported regaining ability to identify, locate and count objects.*

* *Nature Medicine* (May 2021)

Pipeline: solid and advanced product portfolio in ophthalmic gene therapy



Notes:

1. These figures are company estimates of the number of LHON-*ND4* incident patients who will be eligible for LUMEVOQ, based on the Phase III enrolment criteria.
2. These estimates are based on epidemiology numbers and do not necessarily reflect the target patient population of the relevant product candidate once it reaches Phase III.

01

LUMEVOQ® in *ND4*-LHON

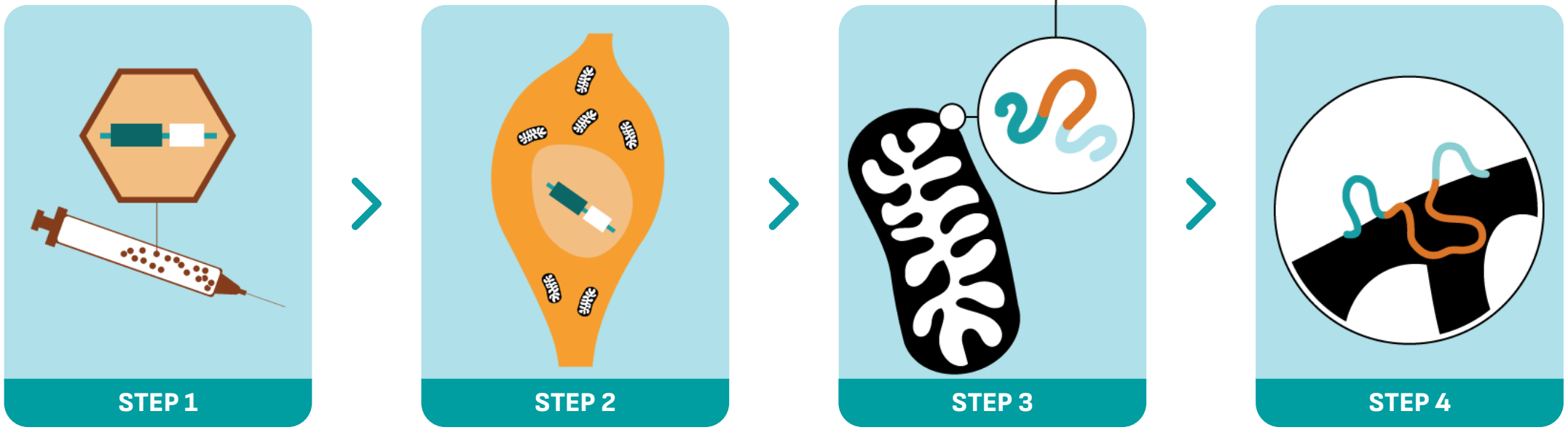
- Therapeutic effect and favorable safety in over 250 patients (Phase I/II, three completed Phase III and one Phase III ongoing)
- UK regulatory submission planned for **H2 2025**
- Additional Phase III for US and EU targeted to start in **H2 2025**

LUMEVOQ® introduces Gene Therapy solution for mitochondrial diseases

Replacing affected mitochondrial mRNA via proprietary MTS* technology

The product of research collaboration with Inserm

MTS in action for LUMEVOQ®:



Retinal cell transduced with vector containing wild-type mitochondrial gene

Wild-type mitochondrial gene transcribed in the nucleus

Wild-type mRNA **delivered by MTS directly** to polysomes located at the mitochondrial surface, where protein synthesis occurs

Finally, the wild-type mitochondrial protein is translocated inside the mitochondrion, where it **restores energy production**

Gene Therapy

MTS (*mitochondrial targeting sequence)

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ND4-LHON: acute, blinding bilateral mitochondrial disease with high unmet medical need

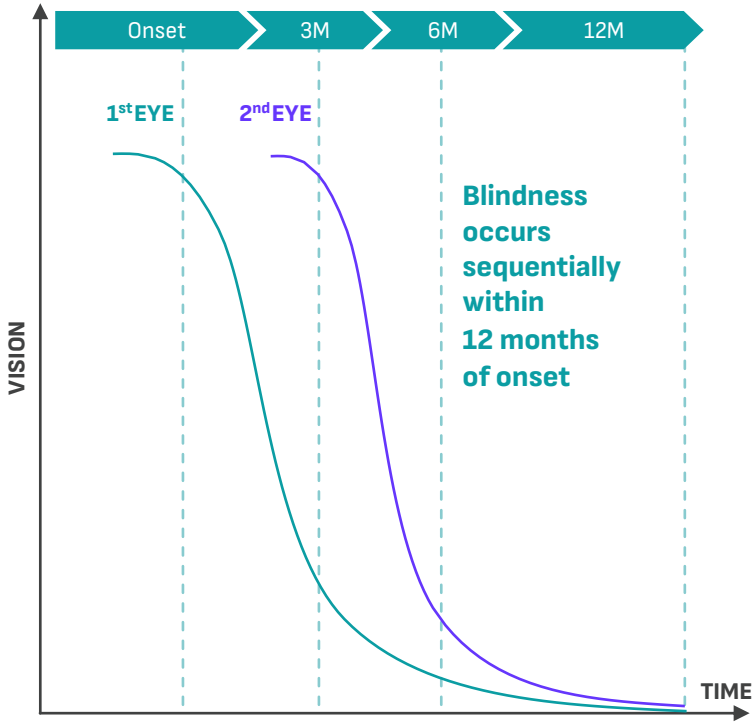
Devastating impact

- Major cause of blindness in young adults
- Causal mutation affects severity: *ND4* (75% of cases) is the most severe mutation with poor visual prognosis

ND4-LHON ncidence	**800–1,000 new patients per year**[1]
Typical age of patient	**15–35 years old**[2]

Acute, rapidly progressing and irreversible: high unmet need

Evolution of vision from onset of disease



"The evolution of natural history eyes … shows an absence of recovery …"

Valerio Carelli et al.

Indirect Comparison of Lenadogene Nolparvovec Gene Therapy Versus Natural History in Patients with Leber Hereditary Optic Neuropathy Carrying the m.11778G>A MT-*ND4* Mutation. Ophthalmol Ther. https://doi.org/10.1007/s40123-022-00611-x

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

1. Based on GenSight analysis of literature and health sector data.
2. Newman NJ, Carelli V, Taiel M, Yu-Wai-Man P. Visual Outcomes in Leber Hereditary Optic Neuropathy Patients With the m.11778G>A (MTND4) Mitochondrial DNA Mutation. *J Neuroophthalmol.* 2020;40(4):547-557.

Clinically meaningful and durable visual function improvement shown in four Phase III studies

REFLECT BILATERAL

98 patients
vision loss ≤ 1 year

Mean change from NADIR* in ETDRS letter equivalents

At 4 years

	1st eye	2nd eye
2 LUMEVOQ® eyes	+ 20	+17
1 LUMEVOQ® eye	+ 19	+14 (placebo)

REVERSE 6-12 MONTHS · RESTORE RESCUE-REVERSE LONG TERM FOLLOW-UP

37 patients
vision loss 6 ≤ 12 months

Mean change from nadir* in ETDRS letter equivalents

At 5 years

LUMEVOQ® eyes	+22
Contralateral eyes (Sham)	+20

RESCUE 0-6 MONTHS · RESTORE RESCUE-REVERSE LONG TERM FOLLOW-UP

39 patients
vision loss ≤ 6 months

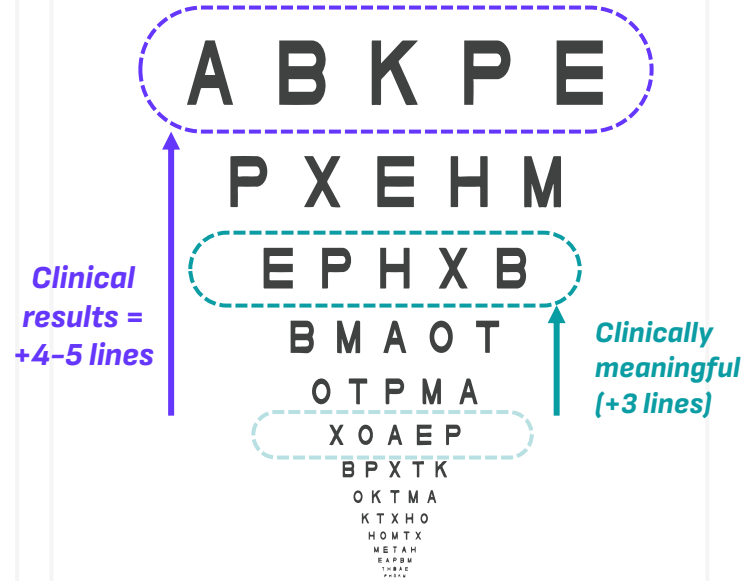
Mean change from nadir* in ETDRS letter equivalents

At 5 years

LUMEVOQ® eyes	+23
Contralateral eyes (Sham)	+18

Note: Improvement in placebo and sham eyes due to a contralateral effect.

Meaningful visual acuity improvement



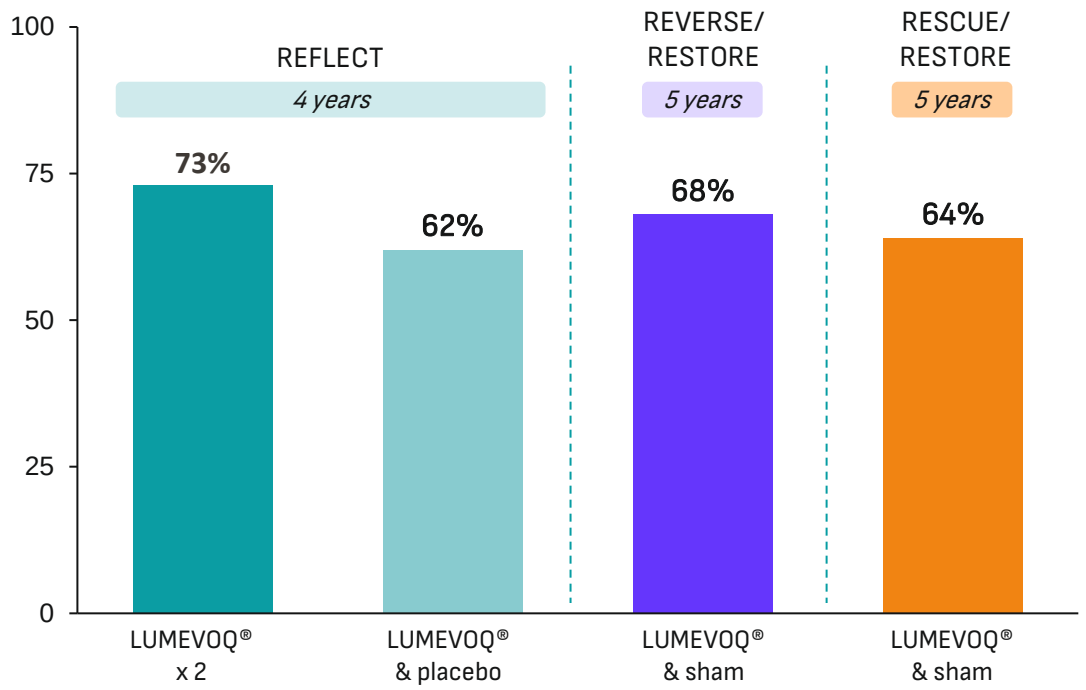
Clinically meaningful improvement of **Quality-of-Life** parameters

*NADIR defined as the **worst** **BCVA** from baseline to time point of interest (5 years for REVERSE and RESCUE, 4 years for REFLECT). Mean change from nadir: last observation for REVERSE/RESTORE and RESCUE/RESTORE (Database lock RESTORE: Jul 4, 2022, completed studies); LOCF imputation for REFLECT (data cut-off Feb 20, 2024, study ongoing). REVERSE: NCT02652780; RESCUE: NCT02652767; RESTORE: NCT03406104; REFLECT: NCT03293524. Data on file; manuscripts in publication review

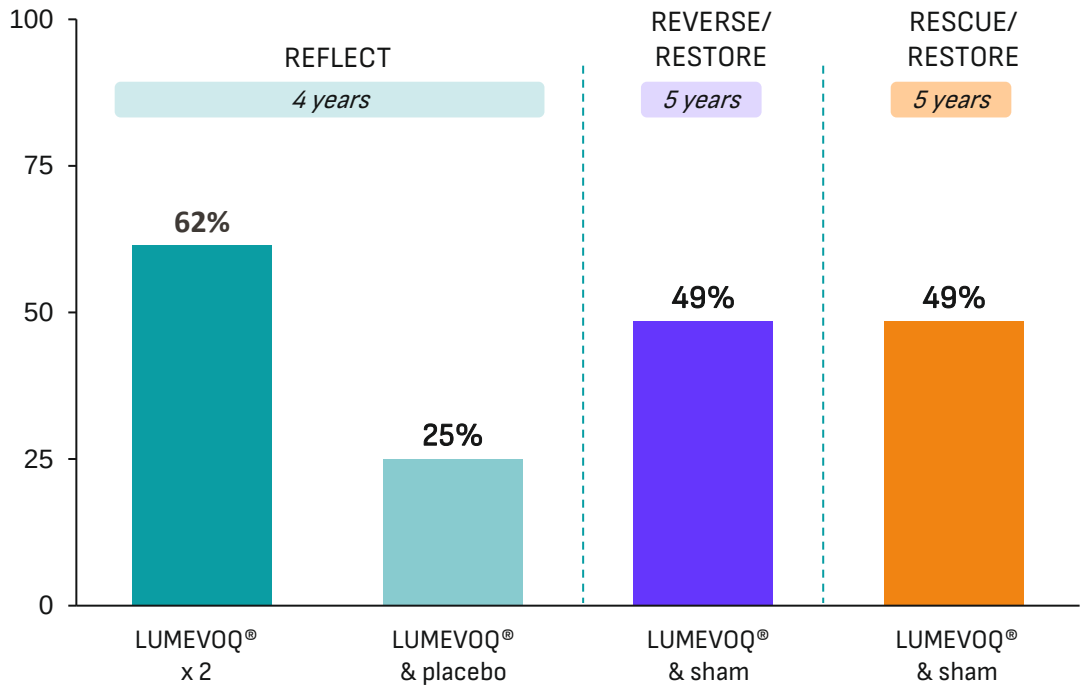
Visual function improvement for the majority of patients

Results not confined to a small number of super-responders

At least +15 letters / 3 lines improvement from nadir



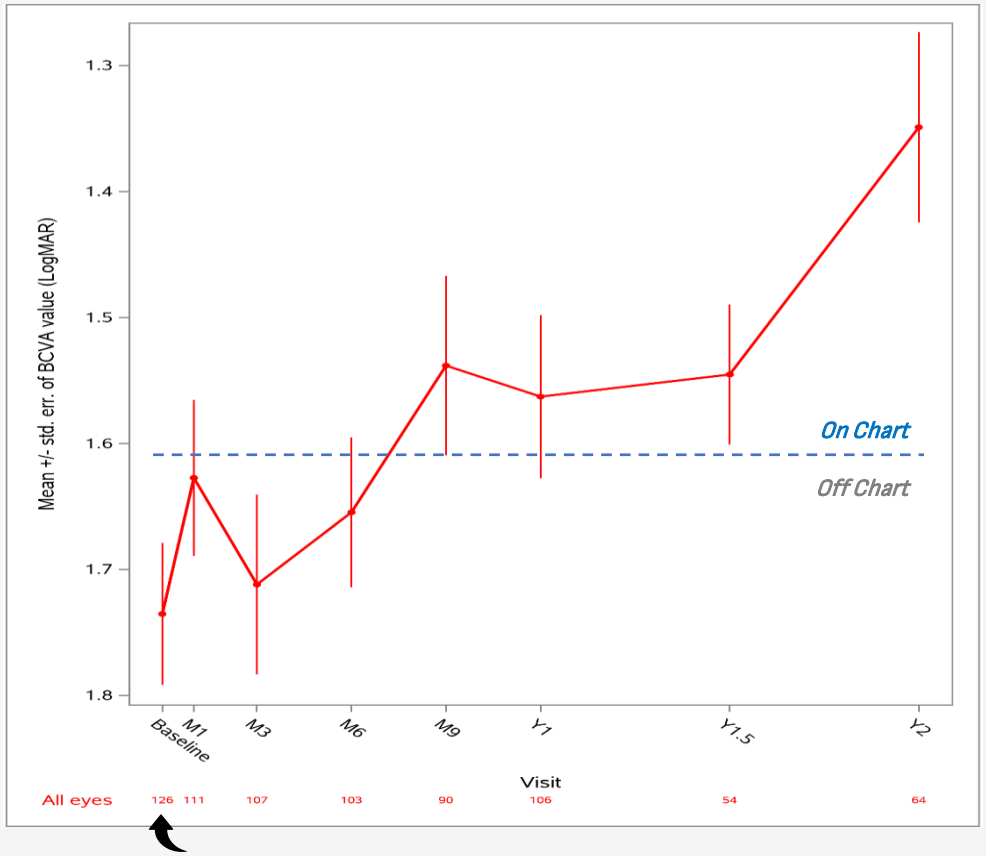
Switch from off-chart to on-chart



*Proportion of patients who responded according to the given definition in either eye at the time point of interest. +15 ETDRS letters = -0.3LogMAR change.
REVERSE: NCT02652780; RESCUE: NCT02652767; RESTORE: NCT03406104; REFLECT: NCT03293524. Results at last observation for REFLECT (data cut-off Feb 20, 2024, study on-going). Results at last observation for REVERSE/RESTORE and RESCUE/RESTORE (Database lock RESTORE: Jul 4, 2022, completed studies.
Data on file; manuscripts in publication review.

LUMEVOQ® real world experience: consistent with clinical trial results

Evolution of mean visual acuity over time (n = 63)



LUMEVOQ® treatment 11.4 months post vision loss on average

Vision function improvement after injection (n=53)

Mean change in visual acuity (vs. nadir)	+21 ETDRS letters
% of eyes with on-chart vision	63.2%
Improvement of at least 15 letters from nadir (%)	61.3%

All patients were treated with LUMEVOQ (63 patients; 126 eyes). Patients treated with idebenone were not excluded from the early access program. At the date of data cutoff, 53 patients (106 eyes) had one year of complete data; 27 patients had 1.5 years of complete data; and 32 patients had 2 years of complete data.

Data cutoff: February 5, 2024

NANOS Conference 2024: Chiara La Morgia et al. Efficacy and Safety of Lenadogene Nolparvovec Gene Therapy for Leber Hereditary Optic Neuropathy in the Real-Life Setting

LUMEVOQ®: favorable safety and tolerability profile

Comprehensive evidence from 252 patients treated in Phase I/II , four Phase III trials and early access

- **No study discontinuations** related to treatment or study procedure[1]
- **Excellent systemic tolerance**, related to the **limited biodissemination**[2]
- **Mostly mild** intraocular inflammation[3], **responsive to conventional treatment**, mostly corticosteroid eye drops alone

REVERSE and **RESCUE**: No prevention of intraocular inflammation: no requirement for oral corticosteroids

REFLECT: Per protocol, for prevention of intraocular inflammation: Oral corticosteroids: 40 mg for 2 days before administration of the gene therapy, 40 mg for the first week after administration, 30 mg for the second week, 20 mg for the third week, and 10 mg for the fourth week.

- **Comparable favorable safety profile for unilateral and bilateral administration**

Notes:

1. No study discontinuation due to ocular adverse events (AEs); no ocular serious AEs (SAEs) in treated eyes (only 1 ocular SAE in a sham eye: retinal tear, unlikely related to treatment/procedure)
2. Negligible in the blood, not detected in the urine and limited and of short duration in the tears
3. The intraocular inflammation was considered likely to be related to the drug and occurred almost exclusively in the anterior chamber and the vitreous.

Safety of Lenadogene Nolparvovec Gene Therapy Over 5 Years in 189 Patients With Leber Hereditary Optic Neuropathy. Catherine Vignal-Clermont et al. AJO 2022. https://doi.org/10.1016/j.ajo.2022.11.026
REVERSE: NCT02652780; RESCUE: NCT02652767; RESTORE: NCT03406104; REFLECT: NCT03293524.

Continuous optimization of manufacturing process for clinical and commercial supply

Corrective & Preventive Actions (CAPA) to close execution gaps in past runs

- Program management expertise (consulting, hirings)
- QC support and expertise
- QA support and expertise
- QC (DP) move to Europe for release of future commercial product

Process improvements in downstream unit operations

- Two successive Drug substance (DS) batches meet all specifications
- Low DS volume required blending of two DS batches to optimize availability of vials for patients
- Drug product fill unit operation successfully transferred to new CDMO with improved recovery

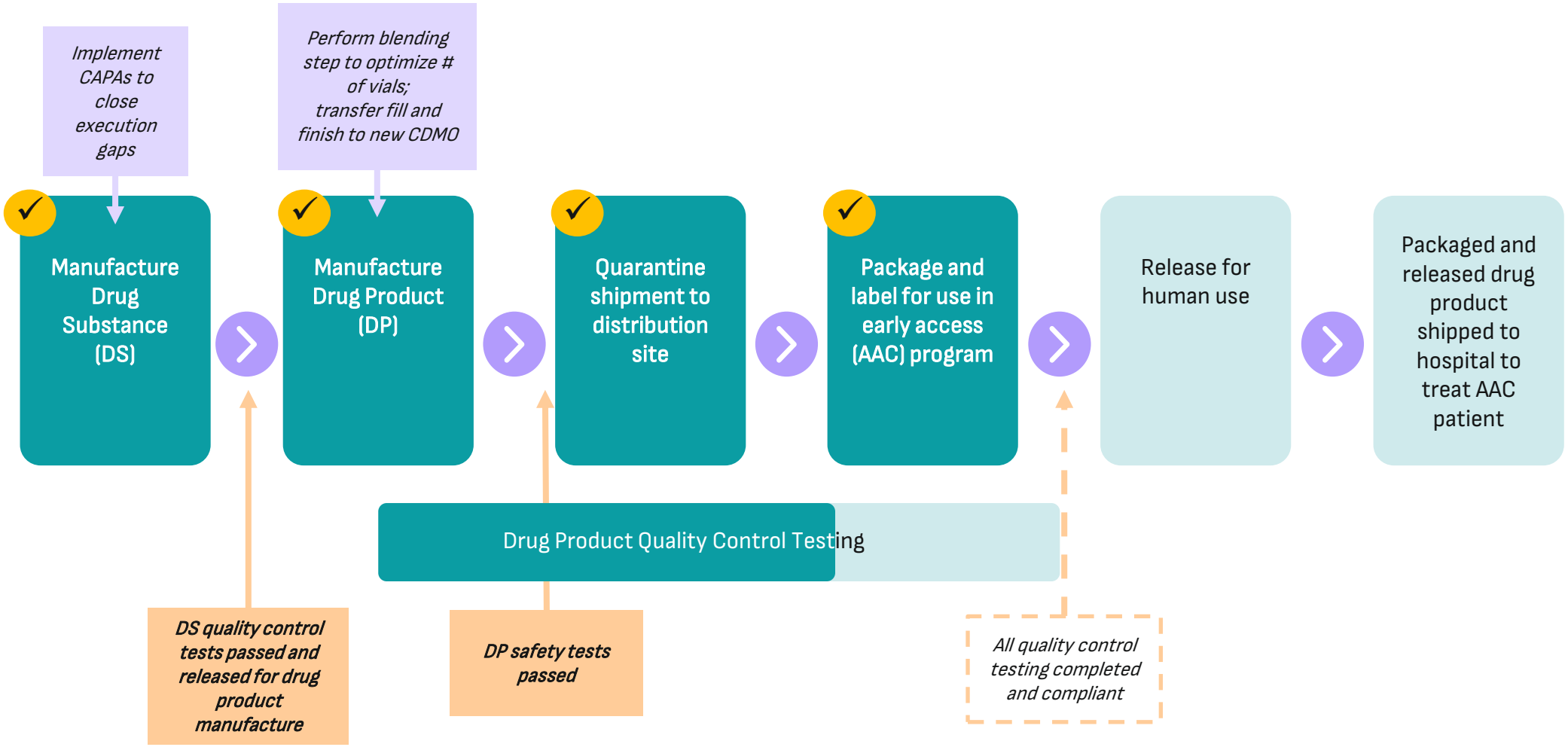
Q3 2024

- Successful blending and fill process to obtain DP for patients in **Early Access Program**
- Positive regulatory feedback on the blending approach (FDA and EMA)

From now to 2025

- Further optimization of manufacturing process for future clinical and commercial supply
- Dual source manufacturing

Manufacturing to enable resumption of paid early access in France



Advancing towards providing access to LUMEVOQ®

Next steps on LUMEVOQ® regulatory path

ansm Agence nationale de sécurité du médicament et des produits de santé	MHRA Regulating Medicines and Medical Devices	EUROPEAN MEDICINES AGENCY	FDA U.S. FOOD & DRUG ADMINISTRATION
• Supply for Early Access program • **"Autorisation d'Accès Compassionnel/Précoce"** or AAC/AAP (former ATU) granted by ANSM – expected to restart in Q4 2024	• Scientific advice meeting outcome (December 2023) confirmed that there will be **no need for another Phase III trial** before the clinical evidence is presented for review	• Defining the next steps for a new MAA • Scientific advice received on new Phase III study RECOVER – positive feedback on overall design • Further Phase III readiness interaction planned	• Scientific advice received on new Phase III study RECOVER – positive feedback on overall design • Further Phase III readiness interaction planned
Restart in Q4 2024	**Submission in H2 2025** **First sales in H2 2026**	**Initiation of RECOVER Phase III in H2 2025**	

Ongoing engagement with patient advocacy groups in Europe and the US

02

GS030

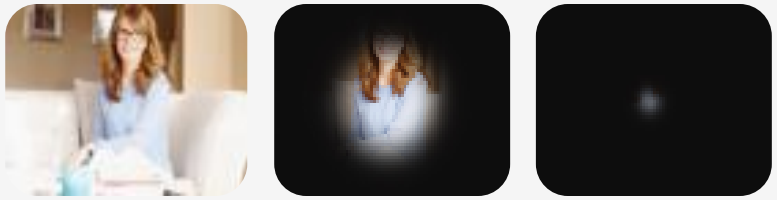
- Optogenetic treatment[1] for photoreceptor degenerative diseases, such as:
  - Retinitis Pigmentosa (RP)
  - Age-Related Macular Degeneration (AMD)
- PIONEER Phase I/II dose escalation study ongoing
- Promising first results published in peer-reviewed journal[2] show fully blind patients regaining ability to locate objects

1. GS030 is a combination treatment consisting of a one-time gene therapy injection and a wearable medical device.

2. Sahel, JA., Boulanger-Scemama, E., Pagot, C. et al. Partial recovery of visual function in a blind patient after optogenetic therapy. *Nat Med* 27, 1223–1229 (2021).

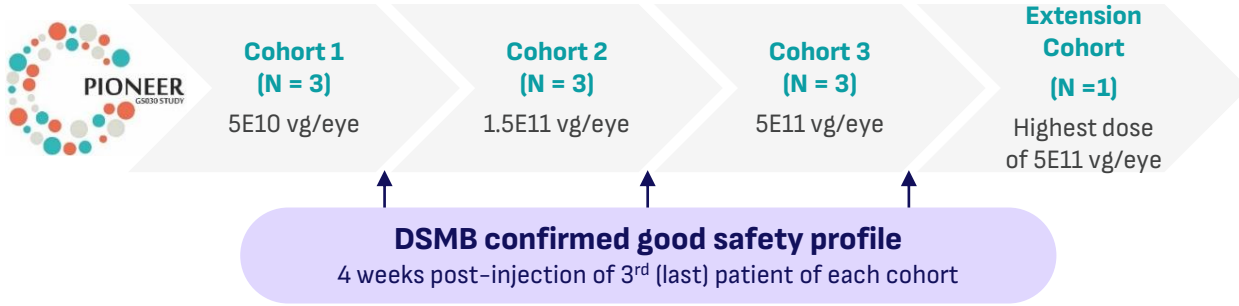
Phase I/II trial (safety and dose finding) for Retinitis Pigmentosa

Retinitis Pigmentosa (RP)



- Blinding genetic disease
- Mutations in over 100 different genes
- Photoreceptor degeneration leads to slow and irreversible progression to blindness, usually reached at age 40-45
- 15-20,000 new patients each year in the US and EU

Study design



- **Phase I/II**, dose-escalation safety study, multi-center (France, UK, US)
- **Study population**: end-stage non-syndromic RP (vision ≤ Counting Fingers)
- Single intravitreal injection in the **worst affected** eye, followed by training on use of ocular device
- Decision to increase the dose of gene therapy taken by a safety board of experts (DSMB)
- **Primary analysis**: Safety at 1 year
- Follow-up of 5 years

Favorable safety profile and promising early efficacy signals from PIONEER

Safety

- **No study discontinuations** related to treatment or study procedure
- **Excellent systemic tolerance**, related to the **limited biodissemination**
- **Mild and moderate intraocular inflammation**, which was **responsive to conventional treatment**, mostly corticosteroid eye drops alone
  - No increased severity at high dose

Per protocol, for prevention of intraocular inflammation:
Oral corticosteroids: 0.5 mg/kg for 1 week before administration of the gene therapy, 1 mg/kg for the first week after administration, 0.5 mg/kg for the second week, 0.25 mg/kg for the third week, and 0.125 mg/kg for the fourth week.

- **Light stimulating goggles (ocular device) well-tolerated**

Efficacy (at one year)

- Vision improvement observed in some patients
  - **Before treatment**: barely able to perceive light
  - **One year after treatment**: ability to locate and count objects
- Best results at the highest dose

Results at one year released in Feb 2023

GenSight Biologics announces 1 Year safety data and efficacy signals from PIONEER Phase I/II clinical trial of GS030, an optogenetic treatment candidate for Retinitis Pigmentosa

Sahel, JA., Boulanger-Scemama, E., Pagot, C. et al. Partial recovery of visual function in a blind patient after optogenetic therapy. Nat Med 27, 1223–1229 (2021). https://doi.org/10.1038/s41591-021-01351-4

03

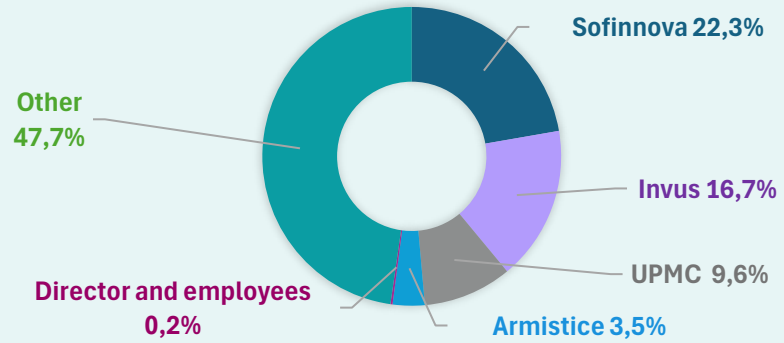
Corporate & Finance

GenSight Biologics financial highlights

Company Overview

Headquarter:	Paris, France
Listing:	Euronext Paris
Tickers:	SIGHT
ISIN:	FR0013183985
Market Cap: (August 30, 2024)	€42,2m
Cash Position: (June 30, 2024)	€6.9 m
Cash runway:	Q3 2025*
Outstanding Shares: (August 30, 2024)	105.6m
Latest Equity Raised: (May 3, 2024)	€9.2m
Equity raised to date: (September 30, 2024)	€218m
IPO Date:	July 13, 2016

Shareholder Structure



Analyst Coverage

CHARDAN	ODDO BHF	Kepler Cheuvreux
Daniil Gataulin (US)	Damien Choplain (FR)	Justine Telliez (FR)

*As of September 30, the Company is financed through mid-November 2024. The Company expects to begin receiving revenues from the restarted AAC program in November 2024. This will extend the cash runway to Q3 2025.

Recapitulation: GenSight Biologics investment case
Gene therapy company with a pivotal stage lead product candidate

Late-stage Biotech company

- **Advanced clinical development stage for lead product** addressing **high unmet medical need** in blinding, irreversible disease
- Well-defined **strategic direction**

Seasoned management team/ Solid investor base

- Management team **delivering on strategy**: manufacturing challenges remediated, paid early access expected to resume in Q4 2024
- **Investor confidence** manifested in two recent rounds of financing

LUMEVOQ® Robust clinical data in LHON

- **Therapeutic effect and favorable safety profile** shown in Phase I/II and Phase III trials and published in peer-reviewed journals
- No further clinical trial required for submission in the UK

LUMEVOQ® Defining registration pathway

- **Paid early access** in France expected to resume in Q4 2024
- **Ongoing discussions** with US FDA, EMA, and UK MHRA to finalize registration pathway

Cutting-edge optogenetics in Retinitis Pigmentosa

- Optionality based on **innovative, mutation-agnostic approach** for photoreceptor degenerative diseases – **promising early results** in the clinic