CORPORATE PRESENTATION
A leading gene therapy biotechnology company

www.gensight-biologics.com
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Corporate Overview

Clinical-stage gene therapy company
- Focused on severe retinal degenerative pathologies leading to blindness as well as CNS diseases
- Well positioned to advance disruptive gene therapy technologies in ophthalmology to commercialization

Two disruptive technology platforms
- Mitochondrial targeting sequence (MTS)
- Optogenetics

Lead projects target:
- GS010 - Leber Hereditary Optic Neuropathy (Phase III)
- GS030 - Retinitis pigmentosa and dry-AMD (Phase I/II)

Listed on Euronext Paris (SIGHT)
- Established in 2012, IPO in July 2016 (EUR45m)
- GenSight Biologics Inc incorporated in the US in May 2017
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

Barrett Katz  
*Chief Medical Officer*

  Prof. of Ophthalmology, Neurology and Neurosurgery
- **DANUBE PHARMA** (2009-2011)  
  CEO
- **FOVEA PHARMA** (2007-2009)  
  CMO
- **EYETECH** (2005-2007)  
  VP of Medical Affairs and Strategy
  MD, Board-certified ophthalmologist & neurologist

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
Degenerative retinal diseases
GenSight targets 3 areas of unmet needs: LHON, RP & DRY AMD

**ELECTRIC SIGNALS ARE SENT TO THE VISUAL CORTEX OF THE BRAIN VIA THE GANGLION CELLS FORMING THE OPTIC NERVE**

**PHOTORECEPTOR CELLS TRANSFORM LIGHT INTO ELECTRIC SIGNALS IN THE EYE**

- Retinitis Pigmentosa (RP)
- Geographic Atrophy (Late stage form of Age-Related Macular Degeneration - AMD)
- Leber Hereditary Optic Neuropathy (LHON)

**Degeneration of RGCs**

**Degeneration of PRs**

*Source: Company*

**NOV 2018 – Non confidential**
1. Genetic disorders and aging are responsible for retinal degenerative diseases that lead to blindness.

2. Therapeutic gene is packaged in a virus vector (AAV). AAV is injected into the eye (intravitreal or subretinal).

3. AAV vector expresses a therapeutic protein in retinal cells. It enables the retina to regain lost function.
Advantages of gene therapy in ophthalmology

THE EYE: STRATEGIC TARGET

- No approved curative treatments for retinal degenerative diseases
- Immune privilege, closed system
- Easy access and ability to get gene to target cells
- Limited number of retinal cells
- Long-term expression of transduced gene due to low turnover rate of retinal cells

AAV: SUCCESSFUL IN RETINA

- 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

NO OTHER APPROVED THERAPEUTIC APPROACHES

- Genetic replacement therapy for diseases caused by single gene mutations (LHON)
- In-situ insertion of therapeutic gene to stimulate sight in patients with severe vision loss due to multiple causes, such as RP and AMD
**Pipeline: solid and advanced product portfolio in ophthalmic gene therapy**

**MTS PLATFORM**

<table>
<thead>
<tr>
<th>Technology</th>
<th>Product Candidate</th>
<th>Indication</th>
<th>Research</th>
<th>Preclinical</th>
<th>Phase I/II</th>
<th>Phase III</th>
<th>Registration</th>
<th>Next Expected Events</th>
</tr>
</thead>
<tbody>
<tr>
<td></td>
<td>GS010 (FDA &amp; EMA Orphan Drug Designation)</td>
<td>LHON ND4</td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
<td>REVERSE: Phase III top-line data reported in April 2018</td>
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<td>RESCUE: Phase III top-line data expected in Q1 2019</td>
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<td>REFLECT*: Phase III recruitment ongoing, top-line data expected in Q2 2020</td>
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<tr>
<td></td>
<td>GS011</td>
<td>LHON ND1</td>
<td></td>
<td></td>
<td></td>
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<td></td>
<td>Initiate preclinical studies following GS010 Phase III clinical data</td>
</tr>
<tr>
<td>Undisclosed Mitochondrial Target</td>
<td>Undisclosed</td>
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</table>

**OPTOGENETICS**

<table>
<thead>
<tr>
<th>Technology</th>
<th>Product Candidate</th>
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<th>Next Expected Events</th>
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<tbody>
<tr>
<td></td>
<td>GS030 (FDA &amp; EMA Orphan Drug Designation)</td>
<td>RP</td>
<td></td>
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<td></td>
<td></td>
<td>PIONEER: First subject in ongoing Phase I/II clinical trial treated in October 2018</td>
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<td></td>
<td></td>
<td></td>
<td>Report interim data one year after last subject treated</td>
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<tr>
<td></td>
<td>GS030</td>
<td>Dry AMD &amp; Geographic Atrophy</td>
<td></td>
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</tbody>
</table>

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

**Lead candidate, GS010, is expected to be 12 months away from MAA submission in Europe**

Note: Please refer to the 2017 Registration Document for a detailed description of regulatory strategy.
GS010

Fully enrolled ongoing Phase III for our lead product candidate dedicated to Leber Hereditary Optic Neuropathy (LHON)
GenSight’s proprietary gene sequencing encapsulated in AAV

The only technology that permits missing mitochondrial proteins to be actively shuttled into the mitochondrion to restore energy production.
LHON: the most common mitochondrial disease causing bilateral blindness at the prime of life

- Incidence: 0.15/100,000
- Prevalence: 1/31k-40k
- Blindness: 15-35y

- Orphan maternally inherited mitochondrial disease
- Painless sudden loss of central vision in the 1st eye with 2nd eye sequentially impaired: symmetric disease with poor visual recovery
- Thinning of the Ganglion Cell Layer occurs after the onset of vision loss and stabilizes at approximately 6 months
- 97% of patients have bilateral involvement < 1 year / 25% of cases are simultaneous
- Targets ND4 which accounts for ~75% of LHON in North America & Europe
RESCUE & REVERSE Phase III trials: time based strategy

**Phase III Trials**

**RESCUE**
- Onset of disease ≤ 6 months
- Initiation: 4Q 2015 (1st patient in February 2016)
- 39 patients in RESCUE (recruitment completed in July 2017)
- Randomized (one eye treated vs. sham), double-masked, sham-controlled, multi-center

**REVERSE**
- Onset of disease 6 months to ≤ 1 year
- 37 patients in REVERSE (recruitment completed in February 2017)

**Endpoints at 48 weeks**

**Primary:**
- Mean Difference in ETDRS of treated eyes compared to eyes receiving sham injection (LogMAR used for statistical analysis)

**Secondary:**
- Measure vision gain, vision stabilization, or reduction in vision decline
- Best or worst eyes vs. sham
- Responders analysis:
  - Gain from baseline of 15 or more ETDRS letters
  - Snellen acuity > 20/200
- SD-OCT, visual field, color and contrast vision
A clinically meaningful improvement of +15 ETDRS letters reported in treated eyes
A continuous bilateral improvement from Baseline to Week 72
**REVERSE** Data at 72 Weeks
Clinically meaningful improvement of contrast sensitivity

- **Contrast sensitivity** as determined by Pelli-Robson low contrast testing increased in both eyes from baseline to week 72:
  
  **Treated eyes:** +0.21 LogCS  
  **Untreated eyes:** +0.15 LogCS

- Proportion of treated eyes that achieved a **clinically meaningful improvement of at least 0.3 LogCS** was statistically significantly higher than that of sham-treated eyes:
  
  **Treated eyes:** 45.9%  
  **Untreated eyes:** 24.3%  
  
  **p=0.0047**
SD-OCT demonstrated statistically significant relative preservation of both retinal ganglion cells and retinal fiber layer in treated eyes vs. untreated eyes.

- **Change in retinal ganglion cell macular volume measured from baseline to week 72:**
  
  **Treated eyes:** no loss  
  **Untreated eyes:** -0.044mm³  
  \[ p = 0.0060 \]

- **Change thickness of the temporal quadrant and papillomacular bundle of the retinal nerve fiber layer from baseline to week 72:**
  
  **Treated eyes:** -1.6 μm  
  **Untreated eyes:** -3.6 μm  
  \[ p = 0.0521 \]

- Sustained preservation of LHON-relevant retinal anatomy in treated eyes further demonstrates the neuroprotective effect of GS010.
REVERSE Data at 72 Weeks
Favorable safety and tolerability profile

- GS010 reported to be well-tolerated
- Ocular adverse events most frequently reported in the therapy group were mainly related to the injection procedure
- Occurrence of intraocular inflammation (accompanied by elevation of intraocular pressure in some patients) responsive to conventional treatment and without sequelae
- No withdrawals from the trial
REFLECT Phase III trial: bilateral treatment

Phase III Trial

- **Initiation**: 4Q 2017 (1st patient treated in March 2018)
- **90 patients** planned (45 in each group) with vision loss ≤ 1 year
- Randomized (two eyes treated vs. one eye treated + placebo in the other eye), double-masked, placebo-controlled, multi-center
- Conducted under a Special Protocol Assessment (SPA) from the FDA

Endpoints at 48 weeks

**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

Confirmatory Phase III study to assess safety and efficacy of a bilateral injection of GS010
GS010: an accelerated path to market

Objective: European marketing authorization submission for GS010 in Q4 2019

(1) FDA approval is expected to be conditional upon the initiation of a trial to evaluate bilateral dosing. Current discussions with the FDA remain ongoing. Please refer to the 2016 Registration Document for a detailed description of regulatory strategy.
Second lead product candidate targeting photoreceptor degenerative diseases (RP/AMD)
RP / AMD: degenerative diseases of photoreceptors leading to blindness

### Retinitis Pigmentosa (RP)
- Blinding genetic disease with multiple mutations (+100 genes)
- Sequential photoreceptor degeneration
- Slow & irreversible evolution leading to blindness

### Age-Related Macular Degeneration (AMD)
- Onset of AMD: 55 to 60 years of age
- Early form: dry-AMD that evolves with aging to late AMD
- Late AMD can either be:
  - Neovascular form (wet-AMD)
  - Geographic atrophy
- Prevalence of geographic atrophy increases with age from 3.5% over 75 years to 22% over 90 years

### Incidence and Prevalence

<table>
<thead>
<tr>
<th></th>
<th>RP</th>
<th>AMD</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>Incidence</strong></td>
<td>15K-20K / year</td>
<td>350k – 400k / year</td>
</tr>
<tr>
<td><strong>Prevalence</strong></td>
<td>350K-400K (1.5 M worldwide)</td>
<td>1.47% with 0.81% geographic atrophy in at least one eye</td>
</tr>
<tr>
<td><strong>Blindness Occurrence</strong></td>
<td>40-45 years old</td>
<td>250 000 with geographic atrophy accounting from 10 to 20% of blind patients</td>
</tr>
</tbody>
</table>
Optogenetics: gene therapy with photosensitive protein

Transferring a gene encoding light-sensitive protein to retinal ganglion cells to restore photoreceptor function in cells that are still wired to the visual cortex.

Restore photoreceptor function in cells by training RGCs to act as photoreceptors.
GS030: stimulating the eye with light through gene therapy

1. AAV2.7m8 + ChrimsonR
   - Gene therapy
   - Transfer of light sensitive protein

2. Expression in retinal ganglion cells

3. Stimulation with optoelectronic device
   - External visual interface transforms external light stimuli into signal that activates the transduced retinal ganglion cells
GS030: activation and stimulation of retinal ganglion cells, providing visual information to the higher visual centers

Irradiance-firing relationship in monkey retina

Expression of ChrR-tdT in midget cells of monkey perifovea

Light-induced visual evoked cortical responses in rats
GS030 is well tolerated in non-human primates and 590 nm LED light stimulation is safe in *rd1* blind mice

<table>
<thead>
<tr>
<th>Toxicity study of GS030 product in non-human primates (n=32)</th>
</tr>
</thead>
<tbody>
<tr>
<td>Bilateral IVT administration with vehicle vs 7.21x10^{10} VG/eye (low dose) vs 7.84x10^{11} VG/eye (high dose) in 100 µL</td>
</tr>
<tr>
<td><img src="image1" alt="Image" /></td>
</tr>
<tr>
<td><strong>Ophthalmology</strong></td>
</tr>
<tr>
<td>• Dose-dependent ocular inflammation in the anterior segment and vitreous, improving/resolving from Month 2 up to Month 6</td>
</tr>
<tr>
<td>• Not associated with any retinal tissue destruction or functional changes</td>
</tr>
<tr>
<td>• No or very slight residual inflammation in all animals at 6 months (self-resolution, no treatment before or after injection)</td>
</tr>
<tr>
<td><img src="image2" alt="Image" /></td>
</tr>
<tr>
<td><strong>Histopathology</strong></td>
</tr>
<tr>
<td>• Eye tissues: dose-dependent minimal mononuclear cell infiltration</td>
</tr>
<tr>
<td>• Other tissues: no histological findings</td>
</tr>
<tr>
<td><img src="image3" alt="Image" /></td>
</tr>
<tr>
<td><strong>Immunogenicity (anti-AAV2 NAb)</strong></td>
</tr>
<tr>
<td>• Expected humoral immune response in serum starting at Day 15, tended to decrease at Week 13 then sustained up to Month 6</td>
</tr>
<tr>
<td>• Dose-dependent local immune response in aqueous humor and vitreous</td>
</tr>
<tr>
<td><img src="image4" alt="Image" /></td>
</tr>
</tbody>
</table>

<table>
<thead>
<tr>
<th>Local tolerance of GS030 product avec light exposure in <em>rd1</em> blind mice (n=36)</th>
</tr>
</thead>
<tbody>
<tr>
<td>Bilateral IVT administration with vehicle vs 7.84x10^{11} VG/eye in 1 µL</td>
</tr>
<tr>
<td>590 nm LED light at 1.4x10^{16} vs 1.7x10^{17} photons/cm²/s vs ambient room light</td>
</tr>
<tr>
<td><img src="image5" alt="Image" /></td>
</tr>
<tr>
<td><strong>Local tolerance (Ophthalmology &amp; Histology)</strong></td>
</tr>
<tr>
<td>• No ophthalmic findings related to GS030-DP or LED light</td>
</tr>
<tr>
<td>• No GS030-DP-related and no LED-related microscopic findings in the retina</td>
</tr>
<tr>
<td>• Transient corneal edema &amp; lens opacity linked to anesthesia procedure</td>
</tr>
<tr>
<td><img src="image6" alt="Image" /></td>
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<tr>
<td><strong>ChrimsonR-tdTomato expression</strong></td>
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<tr>
<td>Good expression of ChrimsonR-tdTomato in retinas and optic nerves</td>
</tr>
</tbody>
</table>

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

**Study design**

- **First-in-man**, dose-escalation safety study, multi-center
- **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

First patient treated in October 2018 at the Moorfields Eye Hospital in the UK
## GS030: CMC progress & Regulatory interactions

### CMC

- **Manufacturing process developed up to 25L**
  - Toxicology batch produced at 25L scale
  - Drug Substance titers (> 2E13 vg/ml) and characteristics in line with expectations
  - Scale up to 100L batch successful

- **Manufacturing process successfully transferred to GMP**
  - GMP clinical supply ready
  - 100L GMP batches manufactured

- **Potency assay**
  - Development completed
  - Transfer in progress

### Regulatory

- **Orphan Drug Designation** granted in the US and in Europe

- **Active strategy & interactions with US and EU Agencies** to obtain advice on preclinical package to support FIM and exploit the existing process of expedited programs

- **CTA approved in the UK and in France**

- **IND released by FDA in the US**
Key expected development milestones for GS030

<table>
<thead>
<tr>
<th>2016</th>
<th>2017</th>
<th>2018</th>
<th>2019</th>
<th>2020</th>
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<tr>
<td>EU ODD</td>
<td>SA EMA</td>
<td>US ODD</td>
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<td>GMP like Batches</td>
<td>GLP Regulatory</td>
<td>Tox Study in Monkeys</td>
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<td>FIM</td>
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<td>Initiation of Pivotal Trials</td>
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</table>

Initiation of Phase I/II in RP patients

Early data from Phase I/II first cohort

Early data from Phase I/II all patients

48 w data from Phase I/II

ODD: Orphan Drug Designation
CTA: Clinical Trial Application
EMA: European Medicines Agency
IDE: Investigational Device Exemption

First patient treated in October 2018 at the Moorfields Eye Hospital in the UK
Building a high strategic value
Curing blindness represents major market opportunity

Favorable reimbursement conditions:

- Gene therapy in ophthalmology for rare diseases could be considered similar to organ transplants for payers
- Blindness imposes a high burden to health systems
  - Total blindness costs exceed tens of billions USD per annum
- Absence of curative treatments
  - Increasing pressure from patients and patients associations

Geographical Split – Blind people in major markets

- 285m people visually impaired
- 39m totally blind
- 6M blind people in North America and EU
- North America 3.2m
- Europe 3.0m
- Asia 16m

Pricing and prevalence: organ transplant / gene therapy

Orphan therapies and transplants: a relevant pricing benchmark

Source: Nature Biotechnology, Volume 33, Number 9, September 2015: The payers' perspective on gene therapy.
Ability to leverage technology platforms and significant expertise to expand the pipeline in ophthalmology and other neurodegenerative disorders.
Financing history

- March 2013 – Series A round – €20m
- June 2015 – Series B round – €32m
- July 2016 – Euronext IPO – €45m
- June 2017 – PIPE – €22m

Listed on Euronext Paris (SIGHT)

- Established in 2012, IPO in July 2016

Recognition from Blue-Chip specialist investors

- Perceptive, Fidelity, Abingworth, Versant, JP MorganAM and others

Analyst coverage

- Oddo & Cie – Pierre Corby (FR)
- Gilbert Dupont – Jamila El Bougrini (FR)
- Chardan – Gbola Amusa (US)
Appendix
A clinically meaningful improvement of +11 ETDRS letters reported in the 37 subjects in both eyes.
REVERSE Topline Data at 48 Weeks
Preservation of the structure of the retina in treated eyes

- SD-OCT demonstrated statistically significant relative preservation of both retinal ganglion cells and retinal fiber layer in treated eyes vs. untreated eyes

  ➢ Change in retinal ganglion cell macular volume measured from baseline to week 48:

    - Treated eyes: no loss
    - Untreated eyes: -0.038 mm³

  ➢ Change thickness of the temporal quadrant and papillomacular bundle of the retinal nerve fiber layer from baseline to week 48:

    - Treated eyes: -0.6 μm
    - Untreated eyes: -3.4 μm
GS010 reported to be well-tolerated

Ocular adverse events most frequently reported in the therapy group were mainly related to the injection procedure

Occurrence of intraocular inflammation (accompanied by elevation of intraocular pressure in some patients) responsive to conventional treatment and without sequelae

No withdrawals from the trial
Contrast sensitivity as determined by Pelli-Robson low vision testing almost doubled in the GS010 treated eyes compared to sham treated eyes from baseline to week 48:

- Treated eyes: +0.20 LogCS
- Untreated eyes: +0.08 LogCS

Post hoc analyses revealed trends that suggest GS010 may have a larger positive impact on the visual acuity of patients at relatively less advanced or severe stages of the disease:

- Subjects who entered study with better vision (on-chart best) tended to have better clinical outcomes. At week 48, in on chart best-seeing eyes, GS010 treated eyes gained on average +12 ETDRS letters (-0.236 LogMAR) compared to +4 ETDRS letters (-0.075 LogMAR) in sham treated eyes.

- Subjects whose vision loss was less than 9 months tended to have better clinical outcomes. 75% of GS010-treated eyes that showed a trend in visual acuity improvement at week 48 had vision loss for less than 9 months at time of treatment administration.

- Subjects who were younger (< 21 years) at enrollment tended to have better clinical outcomes.
Phase I/II: Design & Safety Results

Study design

- First-in-man, dose-escalation safety study, single center (Paris XV-XX)
- Chronic LHON ND4 patients with < 20/200
- Single intra-vitreal injection in the worst affected eye
- Decision to increase the dose taken by a DSMB

Results: Successfully Met Primary Endpoints

- Excellent systemic safety
- No dose-related toxicity
- Mostly mild, well tolerated, ocular side effects that are responsive to standard therapy
- Typical immune responses

Data Safety Monitoring Prior to Dose Escalation
4 weeks post-injection of 3rd (last) patient of each cohort
Phase I/II follow-up
Sustained improvement after 2.5 years in patients with less than 2 years of vision loss

Mean change from Baseline

<table>
<thead>
<tr>
<th>Weeks after administration</th>
<th>Treated Eyes</th>
<th>Untreated Eyes</th>
</tr>
</thead>
<tbody>
<tr>
<td>0</td>
<td>3 (±20)</td>
<td>-</td>
</tr>
<tr>
<td>20</td>
<td>10 (±28)</td>
<td>-</td>
</tr>
<tr>
<td>40</td>
<td>17 (±21)</td>
<td>6 (±20)</td>
</tr>
<tr>
<td>60</td>
<td>29 (±18)</td>
<td>13 (±19)</td>
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<tr>
<td>80</td>
<td>32 (±20)</td>
<td>12 (±23)</td>
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<tr>
<td>100</td>
<td>29 (±24)</td>
<td>15 (±22)</td>
</tr>
<tr>
<td>120</td>
<td>28 (±23)</td>
<td>13 (±21)</td>
</tr>
</tbody>
</table>

Subgroup 1 (n=5)
Vision loss duration ≤ 2 years and Baseline LogMAR ≤ 2.79
(ie excludes “hand motion” patients, in accordance with the Phase III protocol)

Illustration of visual benefit for patient

Source: Company
### Phase I/II follow-up
Sustained improvement after 2.5 years in patients with less than 2 years of vision loss

<table>
<thead>
<tr>
<th>ETDRS letters (LogMAR) Visual Acuity change from baseline Δ TE vs UTE</th>
<th>1.0 year</th>
<th>1.5 year</th>
<th>2.0 years</th>
<th>2.5 years</th>
</tr>
</thead>
<tbody>
<tr>
<td>All patients (n = 14)</td>
<td>+3 letters (-0.06)</td>
<td>+8 letters (-0.16)</td>
<td>+0 letters (-0.00)</td>
<td>+7 letters (-0.14)</td>
</tr>
<tr>
<td>Patients with ≤ 2y disease duration (n = 5)*</td>
<td>+17 letters (-0.34)</td>
<td>+20 letters (-0.40)</td>
<td>+14 letters (-0.28)</td>
<td>+15 letters (-0.30)</td>
</tr>
</tbody>
</table>

Note (*): Excludes “hand motion” patients, in accordance with the Phase III protocol.

### Trends of improved visual acuity in patients with less than 2 years of vision loss
Symptom duration impacts magnitude of treatment effect
• VA beneficial positive trends after 2.5 years in patients ≤ 2y of vision loss with a clinically significant improvement (≥ 15 ETDRS letters)
• Color vision beneficial trends at week 48 in patients with ≤ 2y symptom duration, confirmed with subjective outcome from patients

Baseline vision status at treatment impacts magnitude of treatment effect
• Observed in visual field & color vision tests

Analysis supports protocol strategy for phase III
• Population divided by time from onset
• Effect analyzed on better seeing eye

“Phase 1 Patient”

Now I can see if a traffic light is red or green. In the subway, I can read the names of stations with large letters. I have better autonomy.
Our MTS Sequence enhances growth and ATP synthesis in LHON Fibroblasts

<table>
<thead>
<tr>
<th>Fibroblasts</th>
<th>Survival rate on galactose</th>
<th>Rate of ATP synthesis on galactose</th>
</tr>
</thead>
<tbody>
<tr>
<td>Control</td>
<td>100%</td>
<td>100%</td>
</tr>
<tr>
<td>LHON (mutated ND4)</td>
<td>8%</td>
<td>14%</td>
</tr>
<tr>
<td>LHON + MTS1</td>
<td>12.7%</td>
<td>56%</td>
</tr>
<tr>
<td><strong>LHON + MTS1 &amp; MTS2</strong></td>
<td><strong>54.3%</strong></td>
<td><strong>84%</strong></td>
</tr>
</tbody>
</table>

Both MTS1 and MTS2 sequences are necessary for an efficient transfer of the mitochondrial protein

From patent WO 2006/117250 A2
# GS010 / GS030: IP and market exclusivity timelines

<table>
<thead>
<tr>
<th>Products</th>
<th>Components</th>
<th>Licenses</th>
<th>Associated IP</th>
<th>Patent Term</th>
</tr>
</thead>
<tbody>
<tr>
<td>GS010</td>
<td>Mitochondrial Targeting Sequence</td>
<td>Worldwide exclusive license in ophthalmology</td>
<td>MTS/3'UTR mitochondrial trafficking IP</td>
<td>2026 + PTE/SPC* of 5 years</td>
</tr>
<tr>
<td></td>
<td></td>
<td></td>
<td>Non exclusive license outside of ophthalmology</td>
<td></td>
</tr>
<tr>
<td></td>
<td></td>
<td></td>
<td><img src="https://example.com/inserm.png" alt="Inserm" /></td>
<td></td>
</tr>
<tr>
<td>GS030</td>
<td>Light Sensitive Protein</td>
<td>Worldwide exclusive license in ophthalmology and non-exclusive license outside of ophthalmology</td>
<td>CrimsonR IP</td>
<td>2032 + PTE/SPC* of 5 years</td>
</tr>
<tr>
<td>Engineered AAV</td>
<td>Worldwide exclusive license in Optogenetics</td>
<td>Vector AAV2 7m8 IP</td>
<td></td>
<td></td>
</tr>
</tbody>
</table>

**ORPHAN STATUS - MARKET EXCLUSIVITY**

EU: 10 YEARS + 2 YEARS FOR PEDIATRIC
US: 7 YEARS IN THE US + 6 MONTHS FOR PEDIATRIC

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Note: *Patent Term Extension/Supplementary Protection Certificate*
## 2017 Financial Statements

**P&L (IFRS consolidated)**

<table>
<thead>
<tr>
<th></th>
<th>2016</th>
<th>2017</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>Operating income</strong></td>
<td>3.0</td>
<td>3.7</td>
</tr>
<tr>
<td>Research &amp; Development expenses</td>
<td>(18.5)&lt;sup&gt;(1)&lt;/sup&gt;</td>
<td>(18.7)&lt;sup&gt;(2)&lt;/sup&gt;</td>
</tr>
<tr>
<td>Sales &amp; Marketing expenses</td>
<td>-</td>
<td>(0.8)</td>
</tr>
<tr>
<td>General &amp; Administrative expenses</td>
<td>(6.5)&lt;sup&gt;(1)&lt;/sup&gt;</td>
<td>(8.2)&lt;sup&gt;(2)&lt;/sup&gt;</td>
</tr>
<tr>
<td><strong>Operating profit (loss)</strong></td>
<td>(22.0)</td>
<td>(24.0)</td>
</tr>
<tr>
<td>Financial income (loss)</td>
<td>(0.1)</td>
<td>(0.1)</td>
</tr>
<tr>
<td><strong>Net income (loss)</strong></td>
<td>(22.1)</td>
<td>(24.1)</td>
</tr>
<tr>
<td><strong>Excl. non-cash share-based compensation expenses</strong></td>
<td>(17.4)</td>
<td>(19.3)</td>
</tr>
</tbody>
</table>

**Notes:**
(1) Includes €1.8m and €2.8m of non-cash share-based compensation expenses (IFRS2) in R&D and G&A, respectively, in 2016.
(2) Includes €1.5m and €3.2m of non-cash share-based compensation expenses (IFRS2) in R&D and G&A, respectively, in 2017.
## 2017 Financial Statements

### Balance Sheet (IFRS consolidated)

<table>
<thead>
<tr>
<th></th>
<th>As of December 31</th>
</tr>
</thead>
<tbody>
<tr>
<td></td>
<td>2016</td>
</tr>
<tr>
<td>In million Euros</td>
<td></td>
</tr>
<tr>
<td>Non-current assets</td>
<td>1.2</td>
</tr>
<tr>
<td>Cash and cash equivalents</td>
<td>54.0</td>
</tr>
<tr>
<td>Short term investments</td>
<td>–</td>
</tr>
<tr>
<td>Other current assets</td>
<td>4.1</td>
</tr>
<tr>
<td><strong>TOTAL ASSETS</strong></td>
<td>59.2</td>
</tr>
<tr>
<td>Shareholders’ equity</td>
<td>53.3</td>
</tr>
<tr>
<td>Non-current liabilities</td>
<td>3.0</td>
</tr>
<tr>
<td>Current liabilities</td>
<td>2.9</td>
</tr>
<tr>
<td><strong>Total liabilities</strong></td>
<td>5.9</td>
</tr>
<tr>
<td><strong>TOTAL LIABILITIES AND SHAREHOLDERS’ EQUITY</strong></td>
<td>59.2</td>
</tr>
</tbody>
</table>
## 2017 Financial Statements

Cash Flows Statements (IFRS consolidated)

<table>
<thead>
<tr>
<th>In million Euros</th>
<th>2016</th>
<th>2017</th>
</tr>
</thead>
<tbody>
<tr>
<td>Net cash flows from operating activities</td>
<td>(19.6)</td>
<td>(18.8)</td>
</tr>
<tr>
<td>Net cash flows from investment activities</td>
<td>(0.2 )</td>
<td>(0.7 )</td>
</tr>
<tr>
<td>Net cash flows from financing activities</td>
<td>43.7(1)</td>
<td>20.9(2)</td>
</tr>
<tr>
<td>Increase/(decrease) in cash and cash equivalents</td>
<td>23.9</td>
<td>1.5</td>
</tr>
<tr>
<td>Cash and cash equivalents at the close of the period</td>
<td>54.0</td>
<td>55.4</td>
</tr>
</tbody>
</table>

Notes:

1. Includes €41.4m net proceeds from our Initial Public Offering (IPO) on Euronext Paris in July 2016.
2. Includes €20.7m net proceeds from our Private Placement (PIPE) on Euronext Paris in June 2017.