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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
## Pipeline: solid and advanced product portfolio in ophthalmic gene therapy

<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><strong>MTS platform</strong></td>
<td>GS010</td>
<td>LHON ND4</td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
<td><strong>REVERSE</strong>: Phase III top-line data reported in Apr (48w) &amp; Oct (72w) 2018 and in May 2019 (96w)</td>
</tr>
<tr>
<td></td>
<td>(FDA &amp; EMA Orphan Drug Designation)</td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
<td><strong>RESCUE</strong>: Phase III top-line data reported in Feb (48w), Apr (72w) and Sep (96w) 2019</td>
</tr>
<tr>
<td></td>
<td>GS011</td>
<td>LHON ND1</td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
<td><strong>REFLECT</strong>: Phase III recruitment completed in July 2019, top-line data expected in Q3 2020</td>
</tr>
<tr>
<td></td>
<td>Undisclosed Mitochondrial Target</td>
<td>Undisclosed</td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
<td>Initiate preclinical studies following GS010 Phase III clinical data</td>
</tr>
<tr>
<td><strong>Optogenetics</strong></td>
<td>GS030</td>
<td>RP</td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
<td><strong>PIONEER</strong>: Second cohort ongoing in PIONEER Phase I/II clinical trial. Report interim data one year after last subject treated</td>
</tr>
<tr>
<td></td>
<td>(FDA &amp; EMA Orphan Drug Designation)</td>
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<td></td>
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<td></td>
<td>*Conducting this trial under a special protocol assessment with the FDA</td>
</tr>
<tr>
<td></td>
<td>GS030</td>
<td>Dry AMD &amp; Geographic Atrophy</td>
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</tbody>
</table>

Lead candidate, GS010, is expected to file for MAA in Europe in the coming year
**GS010 aim:** treat LHON, the most common mitochondrial disease causing bilateral blindness in the prime of life

- **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**
- 97% second eye involved within one year
- **Onset on average between age 15-35** (range 1-87 years)
- 80% males
- Incidence 0.15 / 100,000
- Prevalence 1 / 40,000
- 11778-ND4 mutation accounts for ~75% of LHON in USA and Europe


<table>
<thead>
<tr>
<th>Condition</th>
<th>Value</th>
</tr>
</thead>
<tbody>
<tr>
<td>Incidence</td>
<td>0.15/100,000</td>
</tr>
<tr>
<td>Prevalence</td>
<td>1/31k-40k</td>
</tr>
<tr>
<td>Blindness</td>
<td>15-35y</td>
</tr>
</tbody>
</table>
**RESCUE & REVERSE Phase III trials**

**Time-based strategy to assess GS010 efficacy**

**Different patient inclusion criteria**

**REVERSE**
- Onset of disease
  - 6 months to ≤ 1 year
- 37 patients enrolled
- Fully enrolled Feb 2017

**RESCUE**
- Onset of disease
  - ≤ 6 months
- 39 patients enrolled
- Fully enrolled July 2017

**Same design**

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

**Same endpoints at Week 48**

**Primary**
- Mean difference change from baseline, ETDRS letters, drug treated eyes vs. sham treated eyes (LogMAR used for statistical analysis) at Week 48

**Secondary**
- SD-OCT, visual field, color and contrast vision
- Responders analysis:
  - Gain from baseline of 15 or more ETDRS letters
  - Snellen acuity > 20/200
- Treated vs. sham eyes’ BCVA for best-seeing and worst-seeing eyes
- Quality of life assessments
REVERSE: Change from Baseline in BCVA up to Week 96 - LogMAR

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.
REVERSE: Improvement of visual acuity from nadir

Mean change from nadir was calculated using observed values (no data imputation)

<table>
<thead>
<tr>
<th>Change from nadir* in ETDRS letter equivalents</th>
</tr>
</thead>
<tbody>
<tr>
<td>Week 96</td>
</tr>
<tr>
<td>n</td>
</tr>
<tr>
<td>------</td>
</tr>
<tr>
<td>All-GS010 eyes</td>
</tr>
<tr>
<td>All-sham eyes</td>
</tr>
</tbody>
</table>

*Nadir was defined as the worst BCVA from baseline to Week 96
Baseline included

Mean BCVA among GS010-treated eyes and sham-treated eyes evolved with similar trajectories, worsening to a low point, or nadir, before recovering at Week 96 by +28 and +24 ETDRS letters equivalent, respectively.
RESCUE: Time Course LogMAR Visual Acuity up to 96 Weeks

Bilateral improvement of visual acuity after Week 48

LogMAR over Time

<table>
<thead>
<tr>
<th></th>
<th>GS010</th>
<th>Sham</th>
<th>All</th>
</tr>
</thead>
<tbody>
<tr>
<td>Baseline</td>
<td>1.31 (0.52)</td>
<td>1.27 (0.62)</td>
<td>1.29 (0.57)</td>
</tr>
</tbody>
</table>

On-chart

Off-chart
**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.

### Time Course of Best-Corrected Visual Acuity (BCVA) in LogMAR, REVERSE and RESCUE

<table>
<thead>
<tr>
<th></th>
<th>Baseline</th>
<th>GS010</th>
<th>Sham</th>
<th>All</th>
<th>Baseline</th>
<th>GS010</th>
<th>Sham</th>
<th>All</th>
</tr>
</thead>
<tbody>
<tr>
<td>LogMAR All eyes</td>
<td></td>
<td>1.67 (0.50)</td>
<td>1.55 (0.42)</td>
<td>1.61 (0.46)</td>
<td>LogMAR All eyes</td>
<td>1.31 (0.52)</td>
<td>1.27 (0.62)</td>
<td>1.29 (0.57)</td>
</tr>
</tbody>
</table>
**REVERSE & RESCUE: Improvement of visual acuity from nadir**

Visual acuity showed significant, clinically meaningful improvement from worst point of acute phase

<table>
<thead>
<tr>
<th>Change$^1$ from nadir$^2$ in ETDRS letter equivalents</th>
<th>Week 96</th>
</tr>
</thead>
<tbody>
<tr>
<td></td>
<td>n</td>
</tr>
<tr>
<td>All-GS010 eyes</td>
<td>37</td>
</tr>
<tr>
<td>All-sham eyes</td>
<td>37</td>
</tr>
</tbody>
</table>

<table>
<thead>
<tr>
<th>Change$^1$ from nadir$^2$ in ETDRS letter equivalents</th>
<th>Week 96</th>
</tr>
</thead>
<tbody>
<tr>
<td></td>
<td>n</td>
</tr>
<tr>
<td>All-GS010 eyes</td>
<td>34</td>
</tr>
<tr>
<td>All-sham eyes</td>
<td>34</td>
</tr>
</tbody>
</table>

$^1$ Mean change from nadir was calculated using observed values (no data imputation)

$^2$ Nadir was defined as the worst observed BCVA from baseline to Week 96 (baseline included)
Primate Study: Local Biodistribution of Unilaterally Injected GS010

Detection of viral vector DNA in uninjected eye suggests 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

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Notes: One control monkey was injected in one eyes 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.
**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
- 68% of REVERSE subjects attained Clinically Relevant Recovery (CRR) from baseline, compared to 15% in a natural history study
- Patients’ quality of life scores continue to increase, especially in ability to carry out vision-related activities

**RESCUE: 96-Week Follow-Up**

- At Week 96, clinically meaningful bilateral improvement from nadir of BCVA by **+26 ETDRS letters equivalent** in GS010 eyes and **+23 ETDRS letters** in SHAM eyes, maintaining the recovery observed at Week 72
- Results coherent with those of REVERSE: durable bilateral improvement in vision, despite the intervening acute phase
- 63% of RESCUE subjects attained Clinically Relevant Recovery (CRR) from nadir, compared to 28% in a natural history study

- GS010 was well-tolerated through 96 weeks after injection

**FDA End-of-Ph II meeting: Dec 19, 2019**
**EMA Pre-submission meeting: expected early 2020**