Press Release

GenSight Biologics Reports its Cash Position as of September 30, 2019 and Provides Operational Update

Paris, France, October 22, 2019, 7.30 am CEST – GenSight Biologics (Euronext: SIGHT, ISIN: FR0013183985, PEA-PME eligible), a biopharma company focused on discovering and developing innovative gene therapies for retinal neurodegenerative diseases and central nervous system disorders, today reported its cash position as of September 30, 2019, and provided recent operational updates.

Cash and cash equivalents

GenSight Biologics' cash and cash equivalents totaled €5.1 million as of September 30, 2019, compared to €14.3 million as of June 30, 2019. This amount does not include a €4.3 million reimbursement of the 2018 Research Tax Credit, expected in the coming days. Including this impending reimbursement, cash and cash equivalents would be €9.4 million as of September 30, 2019.

The cash burn in the first three quarters of 2019 principally reflects the final steps of pharmaceutical development for GS010 in preparation for a marketing authorization filing in Europe. These are mainly preparatory activities to ensure manufacturing readiness to commercialize under Good Manufacturing Practices (GMP).

"In line with our expectations and including the impending reimbursement of the 2018 Research Tax Credit, we are fully financed until the end of the first quarter of 2020," commented **Thomas Gidoin**, Chief Financial Officer of GenSight Biologics. *"We have been actively working on a financing transaction in the past weeks, favoring less dilutive options, and expect to be able to announce a closing shortly."*

Clinical Development of GS010

Results at 96 weeks of the RESCUE Phase III clinical trial evaluating the efficacy and safety of a single intravitreal injection of GS010 in subjects with LHON due to the ND4 mutation with an onset of vision loss up to 6 months were reported in September 2019. This last set of results showed a durable bilateral improvement in visual acuity of 25 ETDRS letters equivalent versus nadir in GS010-treated eyes. These results from RESCUE show a remarkable correspondence with those from the REVERSE trial, which studied the treatment of subjects at 6 to 12 months after onset of vision loss.

In a natural history study conducted by Santhera[1], 28% of subjects with the G11778A-*ND4* mutation achieved a spontaneous "clinically relevant recovery" (CRR) from nadir in at least one eye. By comparison, 63% of RESCUE subjects achieved this definition of CRR in at least one eye at Week 96, showing a clear superiority to natural history. A similar comparison for REVERSE subjects reveals a much higher rate of CRR (78%) from nadir compared to natural history.

GenSight also reported results from a non-clinical study to investigate the local biodistribution of GS010. In this study, tissue samples from the non-injected eye of monkeys that had been unilaterally injected with GS010 were found to contain GS010 DNA three months after injection, indicating the expression of the

[1] Magda et al (2019), "Natural History of Leber's Hereditary Optic Neuropathy (LHON): Findings from a Large Patient Cohort", Poster presented at NANOS March 16-21, 2019; Poster Session II: Scientific Advancements; Poster: 163

therapeutic gene in the uninjected contralateral eye. These findings support the mechanism for bilateral visual improvement with unilateral GS010 gene therapy, which was consistently observed in LHON subjects in the REVERSE and RESCUE Phase III trials.

Regulatory Pathway of GS010

GenSight is planning to schedule a pre-submission meeting with the EMA in early 2020 and expects to submit an application for marketing approval in Europe in the third quarter of 2020.

An End of Phase II meeting with the U.S. Food and Drug Administration (FDA) was requested as planned, which the agency has now confirmed for December 19, 2019.

"We strongly believe that the remarkable results from REVERSE and RESCUE, showing a clinically significant and durable bilateral improvement of vision, and demonstrating clear superiority to natural history, are a compelling core for our marketing authorization application in Europe," commented **Bernard Gilly**, co-founder and Chief Executive Officer of GenSight Biologics. *"The demonstration of GS010 DNA presence in the uninjected contralateral eye now provides a powerful scientific rationale to support bilateral improvement. We are eager to meet with regulatory agencies in the next few weeks to discuss these major findings."*

Number of outstanding shares

As of September 30, 2019, GenSight Biologics' number of outstanding shares was 29,028,291 ordinary shares.

GenSight Biologics will report its cash position as of December 31, 2019 on January 21, 2020.

GenSight will host a conference call today, October 22, 2019, at 9:30am CEST in French, and at 2.30pm CEST (8.30am EST) in English, to further discuss this operational update.

Conference call in French

Dial-in numbers:

France: +33 (0) 1 7037 7166
Password: GenSight Français

Conference call in English

Dial-in numbers:

United States: +1 212 999 6659
France: +33 (0) 1 7037 7166
United Kingdom: +44 (0) 20 3003 2666
Password: GenSight English

A replay of the calls will be available using the following dial-in numbers:

France: +33 (0) 1 7037 7166
United Kingdom (standard international access): +44 (0) 20 3003 2666
Code: 5645038#

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About GenSight Biologics

GenSight Biologics S.A. is a clinical-stage biopharma company focused on discovering and developing innovative gene therapies for retinal neurodegenerative diseases and central nervous system disorders. GenSight Biologics' pipeline leverages two core technology platforms, the Mitochondrial Targeting Sequence (MTS) and optogenetics to help preserve or restore vision in patients suffering from blinding retinal diseases. GenSight Biologics' lead product candidate, GS010, is in Phase III trials in Leber Hereditary Optic Neuropathy (LHON), a rare mitochondrial disease that leads to irreversible blindness in teens and young adults. Using its gene therapy-based approach, GenSight Biologics' product candidates are designed to be administered in a single treatment to each eye by intravitreal injection to offer patients a sustainable functional visual recovery.