Press Release

GenSight Biologics receives MHRA approval to initiate Phase I/II PIONEER clinical trial of GS030 gene therapy in Retinitis Pigmentosa

- First-in-man application of optogenetics combined with a stimulation device

Paris, France, January 10, 2018, 7.30 am CET – 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, announced UK Medicines and Healthcare Regulatory Agency (MHRA) acceptance of the Company's Clinical Trial Application (CTA) to initiate the PIONEER Phase I/II study of GS030 in patients with Retinitis Pigmentosa (RP).

PIONEER is a first-in-man, multi-center, open label dose-escalation study to evaluate the safety and tolerability of GS030 in subjects with Retinitis Pigmentosa. GS030 is the combination of a gene therapy (GS030-DP) administered via a single intravitreal injection and a wearable optronic visual stimulation device (GS030-MD).

Eligible patients will be those affected by end-stage non-syndromic Retinitis Pigmentosa with vision not better than "counting fingers".

As per protocol, three cohorts of three subjects each will be administered an increasing dose of GS030-DP via a single intravitreal injection in their worse affected eye. A fourth extension cohort will receive the highest tolerated dose. An independent Data Safety Monitoring Board (DSMB) will review safety data of all treated subjects in each cohort and make recommendations before moving to the next higher dose.

The primary outcome analysis will be the safety and tolerability at one year post-injection. GenSight expects to treat the first patient in the United Kingdom in the first quarter of 2018.

Bernard Gilly, CEO and co-founder of GenSight, commented, *"I am particularly excited to have GS030 moving into the clinic. This represents a major milestone for the Company. For the first time ever in ophthalmology, an optogenetic approach combined with a medical device will be tested in humans. If proven safe and effective, this therapy could be transferable from Retinitis Pigmentosa to dry-AMD"*.

GS030 was granted Orphan Drug Designation in the U.S. and Europe. GenSight is in active dialogue with global regulatory agencies and intends to file additional IND and CTA submissions in 2018.

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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.

About GS030

GS030 leverages GenSight's optogenetics technology platform, a novel approach to restore vision in patients by using gene therapy to introduce a gene encoding for a light-sensitive protein into specifically targeted cells of the retina by a single injection in order to make them responsive to light. An external wearable medical device to specifically stimulate the transduced cells is developed to amplify the light signal and further enable vision. Patients will need to wear the external wearable device to enable optimal restoration of visual function. Using this optogenetics technology platform, and with the support of the Vision Institute in Paris, GenSight is developing its second product candidate, GS030, to restore vision in patients suffering from Retinitis Pigmentosa, or RP. GenSight's optogenetics technology platform is independent of the specific genetic mutations that lead to this family of disease. It is expected that GS030 would benefit patients from the early stages of RP. This technology offers the possibility of application to other diseases of the retina where photoreceptors have degenerated, and may be transferable to the dry form of Age Related Macular Degeneration (dry-AMD).

About Optogenetics

Optogenetics is a biological technique that involves the transfer of a gene encoding for a light sensitive protein to cause neuronal cells to respond to light stimulation. As a result, it is a neuromodulation method that can be used to modify or control the activities of individual neurons in living tissue and even in-vivo, with a very high spatial and temporal resolution. Optogenetics combines the use of gene therapy methods to transfer a gene into target neurons with the use of optics and electronics (optronics) to deliver the light to the transduced cells. Optogenetics is widely used by research laboratories throughout the world and holds clinical promise in the field of vision impairment or degenerative neurological disorders.

About Retinitis Pigmentosa (RP)

Retinitis Pigmentosa (RP) is a family of orphan genetic diseases caused by multiple mutations in numerous genes involved in the visual cycle. Over 100 genetic defects have been implicated. RP patients generally begin experiencing vision loss in their young adult years, with progression to blindness by age 40. RP is the most widespread hereditary cause of blindness in developed nations, with a prevalence of about 1.5 million people throughout the world. In Europe and the United States, about 350,000 to 400,000 patients suffer from RP, and every year between 15,000 and 20,000 new patients with RP lose sight. There is currently no existing curative treatment for RP.