

Sensorion announces positive preliminary preclinical data from its Otoferlin gene therapy program

- *In vivo experiments conducted safely in non-human primates show promising preliminary data on inner ear tissue tropism and the achievement of a high transduction rate efficiency*

Montpellier, June 9, 2020 – Sensorion (FR0012596468 – ALSEN) a pioneering clinical-stage biotechnology company which specializes in the development of novel therapies to restore, treat and prevent within the field of hearing loss disorders, announces promising new preliminary data in non-human primates from its ongoing gene therapy program targeting the Otoferlin encoding gene (OTOF).

The following results relate to the first of two preclinical programs conducted under a broad research partnership with Institut Pasteur (Paris). The partnership aims to develop gene therapies to correct, safely and efficiently, hereditary monogenic forms of deafness. The first program targets specifically deafness caused by a mutation of the gene encoding for Otoferlin, which is highly present in cochlear sensory cells and is critical for hearing.

The green fluorescent protein (GFP) was used as an intracellular reporter molecule to assess gene transfer and expression. The selected adeno-associated virus (AAV), a safe and effective vector for gene therapy, enabled expression of the GFP protein under control of the selected promoter. An intense fluorescence due to GFP was detected by immunostaining in the cochlear tissue of two out of three treated animals, demonstrating adequate tropism for inner ear tissue. Remarkably, the expression of GFP was mainly robust in the inner hair cells, indicating an effective transduction of the relevant target cells at levels compatible with future therapeutic intervention of Otoferlin deficiency by gene therapy. These results are expected to be published.

Géraldine Honnet, Chief Medical Officer Sensorion, observes: *“One of the challenges in gene therapy is the precise and safe delivery of the therapeutic to the targeted tissue. In this non-human primate study, the surgical procedure went smoothly in three animals. No infections were observed, either pre- or post-operation, and a perfect scar was obtained. There were no side effects such as transient vertigo resulting from the procedure.”*

“The data confirms that the OTOF program is very promising and represents an important step forward to successfully achieving our gene therapy development to treat hereditary monogenic forms of hearing loss. Our collaboration with one of the leading academic institutions in the field of hearing as well as with one of the top pediatric ENT hospitals in the world sets a solid foundation to create a robust ecosystem in gene therapy development for hearing loss. The preclinical data further validates the potential of our collaboration to develop treatments that could revolutionize the lives of patients with disabling genetic inner ear diseases,” remarked Nawal Ouzren, CEO Sensorion.

Sensorion’s exclusive agreement is with the Genetics and Physiology of Hearing Unit at Institut Pasteur, led by Professor Petit, whose laboratory has developed world-class expertise over the last 25 years in the molecular physiology and pathophysiology of the hearing system and has generated data supporting the development of potential gene therapies. The agreement grants Sensorion exclusive rights to obtain the full intellectual property licenses to develop and market drug products in gene therapy coming from collaborative projects for the restoration, treatment, and prevention of hearing disorders. The French government awarded up to €9.7 million to the “AUDINNOVE” consortium, which includes Necker Hospital, Institut Pasteur and Sensorion, to support the development of the OTOF program into human clinical trials.

About Sensorion

Sensorion is a pioneering clinical-stage biotechnology company, which specializes in the development of novel therapies to restore, treat and prevent within the field of hearing loss disorders. Its clinical-stage portfolio includes one Phase 2 product:

Press release

SENS-401 (Arazasetron) for sudden sensorineural hearing loss (SSNHL). Sensorion has built a unique R&D technology platform to expand its understanding of the pathophysiology and etiology of inner ear related diseases enabling it to select the best targets and modalities for drug candidates. The Company is also working on the identification of biomarkers to improve diagnosis of these underserved illnesses. In the second half of 2019, Sensorion launched two preclinical gene therapy programs aiming at correcting hereditary monogenic forms of deafness including Usher Type 1 and deafness caused by a mutation of the gene encoding for Otoferlin. The Company is uniquely placed, through its platforms and pipeline of potential therapeutics, to make a lasting positive impact on hundreds of thousands of people with inner ear related disorders, a significant global unmet medical need.

www.sensorion-pharma.com

Contacts

CEO

Nawal Ouzren

contact@sensorion-pharma.com

+33 4 67 20 77 30

Financial Communication

LifeSci Advisors - Sophie Baumont

sophie@lifesciadvisors.com

+33 6 27 74 74 49

Label: **SENSORION**
ISIN: **FR0012596468**
Mnemonic: **ALSEN**



Disclaimer

This press release contains certain forward-looking statements concerning Sensorion and its business. Such forward looking statements are based on assumptions that Sensorion considers to be reasonable. However, there can be no assurance that such forward-looking statements will be verified, which statements are subject to numerous risks, including the risks set forth in the 'Document de référence' registration document filed with the 'Autorité des Marchés Financiers' (AMF French Financial Market Authority) on September 7th, 2017 under n°R.17-062 and to the development of economic conditions, financial markets and the markets in which Sensorion operates. The forward-looking statements contained in this press release are also subject to risks not yet known to Sensorion or not currently considered material by Sensorion. The occurrence of all or part of such risks could cause actual results, financial conditions, performance or achievements of Sensorion to be materially different from such forward-looking statements.

This press release and the information that it contains do not constitute an offer to sell or subscribe for, or a solicitation of an offer to purchase or subscribe for, Sensorion shares in any country. The communication of this press release in certain countries may constitute a violation of local laws and regulations. Any recipient of this press release must inform oneself of any such local restrictions and comply therewith.