Press release

Sensorion announces third gene therapy collaboration with Institut Pasteur targeting important pediatric and adult deafness segments

- ***Key new findings by the Institut Pasteur concerning the gene target GJB2, enables Sensorion to potentially address important hearing loss segments in adults and children***
- ***GJB2 mutations are the most prevalent cause of congenital deafness; the gene target potentially opens up a pipeline of indications***
- ***Three initial indications to be targeted: congenital deafness, a progressive form of hearing loss in childhood and early onset of severe presbycusis in adults***
- ***Webcast planned for Tuesday, February 16 at 2:00pm CET***

Montpellier, 15 February, 2021 – 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 the addition of a new gene therapy target, *GJB2 coding for the Connexin 26 protein*, to its development portfolio. The target is the third candidate to emerge from Sensorion's R&D collaboration with Institut Pasteur. The GJB2 program will focus on major new markets with an estimated patient population (prevalence) of 300,000 children and adults in Europe and in the United-States alone.

New research (Boucher *et al*., Proc Natl Acad Sci U S A. 2020;117(49):31278-3128) published by scientists at the Institut Pasteur shows that anomalies in *GJB2* are not only the most common cause of congenital deafness (prevalence of around 200,000 individuals in the US and in Europe alone) but also occur in adult cases of severe age-related hearing loss (around 100,000 adults in the same geographies). Although the types of *GJB2* mutations in children and adults may differ, gene therapy could potentially provide a solution to both.

Sensorion's GJB2 gene therapy program has the potential to target three pathologies related to *GJB2* mutations: age-related hearing loss in adults, progressive forms of hearing loss in children, and pediatric congenital deafness. Initially, the focus will be on the first two populations with GJB2-associated hearing loss, making Sensorion the first company to address these important medical needs in adults and potentially large market opportunities.

Current scientific understanding suggests that mutations in *GJB2* alter a gap junction protein widely expressed in the inner ear, disturbing intercellular exchanges of molecules and leading to hearing loss that is severe-to-profound in a majority of cases.

"The emergence of a new gene therapy target candidate validates our conviction that long-term solutions for restoring hereditary hearing loss will arise from an in-depth analysis of the "genetic landscape" of hearing loss," said **Nawal Ouzren, CEO of Sensorion**. "It was clear that mutations in the GJB2 gene are important in severe to profound childhood hearing loss. However, the new discovery made by our collaborators at Institut Pasteur shows that alteration of this gene in adults offers new opportunities for Sensorion. It marks significant potential expansion of our pipeline and supports our goal of becoming a global leader in the field of gene therapies for hearing loss disorders."

" Until now, the genetics of late forms (age-related deafness or presbycusis) was considered to involve multiple variants in each individual," said **Professor Christine Petit, Director of the French Hearing Institute, an Institut Pasteur Center**. "We have shown that the same genes underlying congenital or childhood deafness are also involved in severe forms of early presbycusis. The presence of these very rare genetic variants makes these forms of presbycusis appear to be monogenic types of hearing loss which can therefore be potentially treated by gene therapy".

Sensorion's collaboration with Institut Pasteur initiated in 2019 has already led to gene therapy candidate programs in two other indications - Otoferlin deficiency and Usher Syndrome Type 1. GJB2-GT is the third program under this collaboration and represents the largest gene therapy opportunity for Sensorion to date.

Considering its broad and rich pipeline, Sensorion may have to consider a reallocation of some resources in the future to focus on the most attractive development programs.

Sensorion will host a webcast on the expansion of its gene therapy pipeline on Tuesday, February 16 at 2:00pm CET (8:00am ET). Please register for the webcast here.

About Sensorion

Sensorion is a pioneering clinical-stage biotech 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: SENS401 (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 aimed 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 potentially 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.
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