Press release

Sensorion receives Orphan Drug Designation for OTOF-GT for the treatment of otoferlin gene-mediated hearing loss from the US Food and Drug Administration

- *Designation follows recent FDA award of Rare Pediatric Disease Designation to Sensorion for OTOF-GT program*
- *Sensorion is on track to file a CTA for OTOF-GT in H1 2023*

Montpellier, November 30, 2022 – 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 that the US Food and Drug Administration (FDA) has granted Orphan Drug Designation (ODD) to OTOF-GT, Sensorion's lead gene therapy program, intended for the treatment of otoferlin gene mediated hearing loss. Sensorion is on track to file a Clinical Trial Application for OTOF-GT in the first half of 2023.

Sensorion's OTOF-GT dual vector AAV gene therapy development program aims to restore hearing in people living with otoferlin deficiency. Patients with mutations in OTOF suffer from severe to profound sensorineural prelingual non syndromic hearing loss. Otoferlin deficiency is responsible for up to 8% of all cases of congenital hearing loss, with around 20,000 people affected in the US and Europe[1].

Sensorion has progressed preclinical and clinical development plans for OTOF-GT and is on track to file a Clinical Trial Application (CTA) in the first half of 2023. In September 2022, the Company received a positive opinion on an application for Orphan Drug Designation (ODD) from the European Medicines Agency (EMA). The European Commission issued the decision on October 11, 2022. On November 7, 2022, Sensorion has also been granted Rare Pediatric Disease Designation to OTOF-GT by the US FDA.

"We are really pleased to have received this significant regulatory feedback from the FDA, following the agency's recent award of Rare Pediatric Disease designation for OTOF-GT. Orphan Drug Designation will support us in advancing our development program, a gene therapy which offers the potential to help patients with a condition for which there are currently no approved curative therapies," said **Géraldine Honnet, Chief Medical Officer of Sensorion**. "We are excited to have achieved this milestone and remain highly focused on the development of our most promising candidates to produce life-changing therapies to restore, treat and prevent hearing loss disorders."

Orphan Drug Designation is granted by the FDA to encourage development of treatment, diagnosis, or prevention of rare diseases, defined as those affecting fewer than 200,000 people in the US. The designation affords Sensorion the potential for certain benefits, including up to seven years of post-approval market exclusivity, assistance in the drug development process, tax credits for clinical development, and exemptions for certain FDA fees.

Under Rare Pediatric Disease designation, a sponsor who receives an approval for a drug or biologic for a "rare pediatric disease" may be eligible for a voucher that can be redeemed to receive priority review of a subsequent marketing application for a different product or sold to another sponsor for priority review of their marketing application, an opportunity for which there is a robust market.

[1] Rodríguez-Ballesteros M, Reynoso R, Olarte M, Villamar M, Morera C, Santarelli R, Arslan E, Medá C, Curet C, Völter C, Sainz-Quevedo M, Castorina P, Ambrosetti U, Berrettini S, Frei K, Tedín S, Smith J, Cruz Tapia M, Cavallé L, Gelvez N, Primignani P, Gómez-Rosas E, Martín M, Moreno-Pelayo MA, Tamayo M, Moreno-Barral J, Moreno F, del Castillo I. *A multicenter study on the prevalence and spectrum of mutations in the otoferlin gene (OTOF) in subjects with nonsyndromic hearing impairment and auditory neuropathy*. Hum Mutat. 2008 Jun;29(6):823-31. doi: 10.1002/humu.20708. PMID: 18381613.

About Sensorion

Sensorion is a pioneering clinical-stage biotech company, which specializes in the development of novel therapies to restore, treat and prevent hearing loss disorders, a significant global unmet medical need.
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 mechanisms of action for drug candidates. Its portfolio combines both small molecule programs and a preclinical portfolio of inner ear gene therapies.
Its clinical-stage portfolio includes one Phase 2 product: SENS-401 (Arazasetron) progressing in a planned Phase 2 proof of concept clinical study of SENS-401 in Cisplatin-Induced Ototoxicity (CIO) and, with partner Cochlear Limited, in a study of SENS-401 in patients scheduled for cochlear implantation. A Phase 2 study of SENS-401 was also completed in Sudden Sensorineural Hearing Loss (SSNHL) in January 2022.
Sensorion pursues its broad strategic collaboration with Institut Pasteur focused on the genetics of hearing. It has two gene therapy programs aimed at correcting hereditary monogenic forms of deafness including OTOF-GT, targeting deafness caused by a mutation of the gene encoding for otoferlin, and hearing loss related to mutation in *GJB2* gene to potentially address important hearing loss segments in adults and children (GJB2-GT). The Company is also working on the identification of biomarkers to improve diagnosis of these underserved illnesses.
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