Press release

Sensorion Submits Clinical Trial Application for Lead Gene Therapy Candidate OTOF-GT in the UK

Montpellier, July 10, 2023, 7.30am CET – 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, today announced that it has submitted a first Clinical Trial Application (CTA) for OTOF-GT to the UK's Medicines and Healthcare products Regulatory Agency (MHRA).

The phase 1/2 clinical trial (Audiogene), aims to evaluate the safety, tolerability and efficacy of intra-cochlear injection of OTOF-GT, for the treatment of otoferlin gene-mediated hearing loss in pediatric patients aged up to 31 months. The CTA submission follows extensive preclinical studies assessing the safety and efficacy of OTOF-GT and successful manufacturing of the gene therapy Drug Product for the clinical trial.

Sensorion's OTOF-GT dual AAV vector gene therapy development program aims to restore hearing in patients with mutations in OTOF who suffer from severe to profound sensorineural prelingual non syndromic hearing loss. Otoferlin is a protein expressed in the inner hair cells (IHC) present in the cochlea and is critical for the transmission of the signal to the auditory nerve. Otoferlin related hearing loss 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]. OTOF-GT previously received Orphan Drug Designation from the US Food and Drug Administration (FDA)[2] and the European Medicines Agency (EMA)[3] and Rare Pediatric Disease Designation from the FDA in Q4 2022.

Nawal Ouzren, Chief Executive Officer of Sensorion, commented: "This first gene therapy CTA filing is a major milestone for our OTOF-GT program and Sensorion's broader gene therapy franchise. This is the first of several planned CTA filings and we hope to commence patient recruitment soon, subject to the CTA approval. We look forward to continuing to work with clinicians, regulatory authorities and patient groups to address unmet and underserved medical needs in the intricate hearing space."

Géraldine Honnet, Chief Medical Officer of Sensorion, added: "There are currently no approved drug for patients with mutations of the gene encoding for otoferlin. Our goal is to transform the standard of care for OTOF newborns, by reducing dependence on cochlear implants, which would transform their quality of life. We believe that gene therapy has the potential to offer permanent solutions to patients with diseases caused by the OTOF mutation and other inner ear diseases and we are really excited to move OTOF-GT forward towards clinical development."

Sensorion plans to submit the CTA in Europe in the coming weeks.

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[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.

[2] FDA Orphan Drug Designations and Approvals https://www.accessdata.fda.gov/scripts/opdlisting/oopd/listResult.cfm

[3] EU Community Register of orphan medicinal products https://ec.europa.eu/health/documents/community-register/html/o2698.htm

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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.
It has two gene therapy programs aimed at correcting hereditary monogenic forms of deafness, developed in the framework of its broad strategic collaboration focused on the genetics of hearing with the Institut Pasteur. OTOF-GT targets deafness caused by mutations of the gene encoding for otoferlin and GJB2-GT targets hearing loss related to mutations in *GJB2* gene to potentially address important hearing loss segments in adults and children. The Company is also working on the identification of biomarkers to improve diagnosis of these underserved illnesses.
Sensorion's portfolio also comprises clinical-stage small molecule programs for the treatment and prevention of hearing loss disorders.
Sensorion's 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.
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