Press release

Sensorion Submits Clinical Trial Application for OTOF-GT, its Lead Gene Therapy Candidate, in Europe

Montpellier, July 19, 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 Clinical Trial Application (CTA) to initiate a Phase 1/2 clinical trial of OTOF-GT in Europe (France, Italy and Germany).

On July 10th, 2023, Sensorion submitted a first 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 administration of OTOF-GT, for the treatment of otoferlin gene-mediated hearing loss in pediatric patients aged from 6 to 31 months. The CTAs 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, Sensorion's Chief Executive Officer, commented: "Our gene therapy programs, OTOF-GT being the most advanced project, offer great hope for many children suffering from congenital deafness, who today cannot benefit from approved curative treatments. With this filing in Europe, we are taking the final steps in preparing for the start of the clinical trial for OTOF-GT and consolidating our position as one of the leading players in the field of gene therapies applied to hearing loss. We are fully focused on the development timetable and on revealing the value of this portfolio of innovative drug candidates, while respecting our commitments to patients, academic, hospital partners and shareholders."

Géraldine Honnet, Chief Medical Officer of Sensorion, added: "We are excited to advance OTOF-GT, our first gene therapy program, to the clinic to address unmet medical need in the complex hearing space. Pending regulatory authority approvals, we are looking forward to recruiting the first patients aged from 6 to 31 months and offer them the potential of an innovative treatment to correct their hearing loss due to otoferlin deficiency. Hearing loss caused by mutations of the gene encoding for otoferlin is a debilitating disorder with currently no approved drugs."

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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

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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