Press release

Sensorion Announces Preliminary Positive Data from the First Cohort of the Audiogene Phase 1/2 Gene Therapy Clinical Trial

2025-07-01

- **SENS-501, including the surgical delivery of the gene therapy, shows a good safety profile in all patients treated so far**
- **Three-month results from a SENS-501 treated toddler in the first cohort demonstrate early promising hearing improvement**
- **The second cohort assessing a higher dose of SENS-501 is ongoing and recruitment is close to being completed**

MONTPELLIER, France--(BUSINESS WIRE)-- Regulatory News:

Sensorion (FR0012596468 – ALSEN) a pioneering clinical-stage biotechnology company specializing in the development of novel therapies to restore, treat and prevent hearing loss disorders, today announced preliminary positive data from the first cohort of the Phase 1/2 Audiogene clinical trial evaluating the low dose of SENS-501, the Company's gene therapy candidate being developed to treat a specific form of congenital deafness linked to mutations in the OTOF (otoferlin) gene. The second cohort at a higher dose is ongoing and recruitment is close to being completed.

The results from all patients dosed to date (5) confirm that SENS-501 and the corresponding surgical procedure are well tolerated by all participating infants and toddlers (aged 6 to 31 months and naive of cochlear implants at the time of the injection, as per study protocol) having received a gene therapy injection. Intracochlear administration of SENS-501 was uneventful, and no serious adverse events or serious side effects were reported.

Three patients were enrolled into Cohort 1 and received a low dose of SENS-501 of 1.5E11 vg/vector/ear, corresponding to the minimally effective dose in preclinical studies. The primary objective is to assess the safety and feasibility of the intra-cochlear administration of SENS-501. In Cohort 1, early signs of hearing improvement

were observed in Patient 3, aged 11 months at the time of injection. The clinical response observed in Patient 3 was evaluated using standard hearing tests carried out by the investigators (Auditory Brainstem Response ABR, Pure Tone Audiometry PTA, and Patient (Parents) Reported Outcomes PROs).

Three-month data from the Patient 3 include:

- Positive ABR responses at two frequencies, with the best frequency reaching 70 dB.
- Improvement of hearing levels across two speech frequencies with best frequency reaching 90 dB level, per PTA.
- Meaningful changes in responses to sounds and voices as reported by the parents with an IT-MAIS score increase of 16 points (145% relative improvement from baseline), and met expected auditory milestones based on an age-based parent questionnaire and according to the patient's age (LittlEARS).

The recruitment in Cohort 2, utilizing a second and higher dose level, is nearly complete. The Company plans to provide the next update when Cohort 2 data have reached sufficient maturity to determine next steps for the program.

Professor Catherine Birman, ENT surgeon, otolaryngologist, and Senior Staff Specialist at the Children's Hospital at Westmead, Australia, commented: "I'm thrilled to report the preliminary Cohort 1 data of SENS-501 in the first infants and toddlers treated with this highly innovative therapy. Treatment with SENS-501 had a good safety profile and the onset of early auditory responses observed in Patient 3 of the first cohort is very encouraging, especially given the very low dose of vector injected, which is primarily intended at assessing the safety of the therapeutic and of the intracochlear surgical procedure. I look forward to Patient 3's next visit and continuing the Audiogene study with the second cohort to assess a higher dose of SENS-501. Treating children under 31 months of age and naive of cochlear implants is a much-needed undertaking, as restoring hearing in the first three years of childhood has the potential to result in de-novo language acquisition. We thank Sensorion for their commitment to this patient population."

The Phase 1/2 clinical trial Audiogene (**ClinicalTrials.gov ID:** NCT06370351), developed in the frame of the strategic partnership with the Institut Pasteur and led by Professor Natalie Loundon, Coordinating Investigator, M.D., Director of the Center for Research in Pediatric Audiology, Pediatric Otolaryngologist and Head and Neck Surgeon, Necker Enfants Malades, AP-HP, in Paris, France, aims to evaluate the safety and efficacy of an intra-cochlear injection of SENS-501 for the treatment of OTOF gene-induced hearing loss in paediatric patients aged 6 to 31 months and naive of cochlear implants at the time of the gene therapy treatment.

Audiogene consists of a dose-escalation part, comprising two cohorts of three patients each, assessing a low dose of SENS-501 in Cohort 1 (1.5E11 vg/vector/ear) and a higher dose of SENS-501 in Cohort 2 (4.5E11 vg/vector/ear).

The dose-escalation part will be followed by a dose-expansion cohort at the selected dose. While safety is the primary endpoint of the dose escalation study, Auditory Brainstem Response, twelve months following the injection, will be the primary endpoint for the dose expansion part. Audiogene is the first gene therapy clinical trial addressing a unique homogeneous population of infants and toddlers (aged 6 to 31 months and naive of cochlear implants at the time of the gene therapy injection). Addressing this young patient population aims at maximizing the chances of these infants and toddlers to acquire language (below three years old, when brain plasticity is optimal). Furthermore, and uniquely to Audiogene's gene therapy program, all enrolled patients should not have current or previous cochlear implantation in the treated or contralateral ear, allowing to best document the contribution of the gene therapy in speech development.

About SENS-501

SENS-501 (OTOF-GT) is an innovative gene therapy program developed to treat a specific form of congenital deafness linked to mutations in the OTOF (otoferlin) gene. This gene plays a key role in the transmission of auditory signals between the hair cells of the inner ear and the auditory nerve. When this gene is defective, affected individuals are born with severe to profound hearing loss.

The aim of SENS-501 (OTOF-GT) is to restore hearing by introducing a functional copy of the OTOF gene directly into hair cells via viral vector technology (AAV). This therapy aims to restore the normal process of converting sound into electrical signals, enabling patients to regain their hearing ability. Currently in the clinical research phase, this gene therapy program represents significant hope for families affected by this rare form of genetic deafness. SENS-501 (OTOF-GT) embodies a commitment to scientific innovation in the field of hearing, with the potential to dramatically improve the quality of life of patients suffering from genetic deafness. This gene therapy for patients suffering from otoferlin deficiency has been developed in the framework of RHU AUDINNOVE, a consortium composed of Sensorion with the Necker Enfants Malades Hospital, the Institut Pasteur, and the Fondation pour l'Audition. The project is partially financed by the French National Research Agency, through the "investing for the future" program (ref: ANR-18-RHUS-0007).

The OTOF gene targeted by the Audiogene trial was discovered in 1999 at the Institut Pasteur, by Prof. Christine Petit's team (Institut reConnect, Institut de l'Audition, Pasteur Institute), who also unraveled the pathophysiology of the corresponding deafness (DFNB9).

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. SENS-501 (OTOF-GT) currently being developed in a Phase 1/2 clinical trial, 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 programs of a clinical-stage small molecule, SENS-401 (Arazasetron), for the treatment and prevention of hearing loss disorders. Sensorion's small molecule progresses in a Phase 2 proof of concept clinical study of SENS-401 in Cisplatin-Induced Ototoxicity (CIO) for the preservation of residual hearing. Sensorion, with partner Cochlear Limited, completed in 2024 a Phase 2a study of SENS-401 for the residual hearing preservation 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.
www.sensorion.com

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 2024 full year report published on March 14, 2025, and available on our website 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.

Investor Relations
Noémie Djokovic, Investor Relations and Communication Associate

ir.contact@sensorion-pharma.com

Press Relations
Ulysse Communication
Bruno Arabian / 00 33(0)6 87 88 47 26
barabian@ulysse-communication.com
Nicolas Entz / 00 33 (0)6 33 67 31 54
nentz@ulysse-communication.com

Source: Sensorion