



Press Release

AudioCure Pharma's AC102 receives EMA orphan drug designation for the treatment of sudden sensorineural hearing loss (SSNHL)

- The EMA's Committee for Orphan Medicinal Products has granted orphan drug designation to AC102, concluding that AudioCure's lead compound may be of significant benefit for patients suffering with the rare and chronically debilitating disease of sudden sensorineural hearing loss (SSNHL).
- Comprehensive preclinical *in vivo* data provide strong evidence of efficacy in a model of acute hearing loss.

Berlin, 29 January 2019 – The German pharmaceutical company AudioCure Pharma GmbH announced today that their first in class molecule AC102 has received orphan drug designation by the European Medicines Agency (EMA) for the treatment of sudden sensorineural hearing loss (SSNHL). The EMA exclusively grants orphan drug designation to medicinal products intended to treat a severely debilitating condition of low prevalence when there is sufficient evidence that the drug has the potential to bring significant benefit to affected patients.

Dr. Reimar Schlingensiepen, CEO of AudioCure commented: "The orphan drug designation is a major milestone in the development program of AC102, enabling a more efficient and cost-effective regulatory pathway, and is therefore an important step in pursuing our goal of developing treatments for hearing disorders with significant unmet medical needs. Preparations for our Phase I clinical trial with AC102 are well underway and we look forward to future interactions with the EMA in our effort to develop our lead compound to the market."

SSNHL is a form of hearing loss with a rapid onset. In most cases the cause is unknown. The prevalence of the disorder is considered rare with 4 out of 10,000 people affected. Prof. Dr. Stefan Plontke, Head of Ear, Nose and Throat Clinic at Martin Luther University, Halle, Germany explained: "Hearing loss affects peoples' quality of life much more than one would expect. Even mild levels of hearing loss can increase the long-term risk of cognitive decline and dementia. Nevertheless, there are no approved drugs on the market for acute hearing loss. As a medical doctor I am in urgent need of a treatment with a proven efficacy and safety profile that I can offer to my patients."

AC102's mode of action is unique in addressing, in parallel, three of the pathological processes that lead to hearing loss: it protects cells and restores functionality of the inner ear not only by preventing cell death of outer hair cells but also by enabling the repair processes of inner hair cells and reducing inflammation¹.



Notes to Editor

About AudioCure

AudioCure Pharma GmbH is a pharmaceutical R&D company headquartered in Germany with a focus on hearing impairments. AudioCure was founded by Professor Hans Rommelspacher, a clinician and academic who has dedicated his career to the discovery and preclinical development of drug candidates. It is Prof. Rommelspacher's scientific expertise and lifelong dedication that led to the discovery of AC102 and its sister compounds. He is joined by Dr. Reimar Schlingensiepen, CEO, who brings to AudioCure over 20 years' experience in medicine and the biopharmaceutical industry in both managerial roles and as an investigator. Highly skilled scientists and executives complete the team. AudioCure's mission is to turn incurable hearing loss into a treatable event.

About AC102

AC102 is a disease modifying novel molecule. Preclinical models have demonstrated the unique potential of AC102 to act beneficially upon three cell types of the inner ear which are critical for the hearing process: the sensory inner hair cells, the sensory outer hair cells and the acoustic nerve¹.

AC102 acts as an antagonist to the multitude of pathological processes leading to hearing loss. Therefore, treatment with AC102 almost completely reverses the reduction of hearing in an *in vivo* model of permanent hearing loss. Furthermore, the compound is still effective when given over an extended time window.

About hearing disorders, SSNHL

Hearing disorders are a global problem with immense consequences for the patients and their families. The loss of communication can lead to social withdrawal, isolation and depression as well as limiting job opportunities.² Hearing loss also presents a significant economic burden, reflected by an estimated loss in gross domestic product of up to 2% in developed countries.³

Nevertheless, to date there is no approved drug for sudden sensorineural hearing loss (SSNHL). This is an alarming condition in which hearing loss occurs rapidly over hours or noticed upon waking, prompting patients to visit a doctor or the emergency clinic. In a number of patients hearing loss can be permanent⁴.

For further information, please visit www.audiocure.com

References

¹AudioCure, data on file

²Mortality and Burden of Diseases and Prevention of Blindness and Deafness. WHO, 2012

³Prevention of noise-induced hearing loss. WHO report, Geneva 1997

⁴Stachler *et al*, (2012) Otolaryngology Head and Neck Surgery 146(1S): 1–35

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