東北大学 TOHOKU UNIVERSITY

Press Release

November 19, 2025

World's First Mitochondrial Disease Treatment 'MA-5' Commences Phase II Clinical Trial

MA-5 is a therapeutic intervention for mitochondrial disease that has been demonstrated to ameliorate severe symptoms such as neurological disorders, renal dysfunction, hepatic dysfunction and motor disorders in animal disease models, which are induced by mitochondrial dysfunction. Crucially, it also improved survival rates. This treatment was developed by a team led by Professor Takaaki Abe at Tohoku University Graduate School of Medicine and Graduate School of Biomedical Engineering.

Mitochondrial diseases affect the function of acellular organelle called the mitochondria, which produces almost all of the energy (ATP) we need to live and function. A lack of ATP caused by mitochondrial dysfunction has many major downstream effects such as systemic organ damage, muscle wasting (sarcopenia), and deafness. With such severe symptoms, finding an effective treatment can help improve the overall prognosis of patients around the world with mitochondrial diseases.

Since results in various mouse hearing loss models (including mitochondrial disease models) and human induced pluripotent stem cell (iPS cell)-derived inner ear cells have demonstrated the efficacy of MA-5 against hearing loss, this promising treatment is moving on to the next stage of testing. Starting in December 2025, the research group will initiate an investigator-initiated Phase II trial of MA-5 for patients with mitochondrial disease accompanied by hearing loss at four medical institutions in Japan (jRCT2031250505). The goal is to obtain approval for MA-5 as a treatment for both mitochondrial disease and hearing loss.

This research is expected to contribute to slowing the progression of mitochondrial disease, a rare and intractable condition for which there are

currently no treatments, and to improving hearing loss, a condition that affects many people.

The research group is led by Professor Takaaki Abe and Professor Kei Murayama from the Department of Pediatrics and Diagnosis and Treatment of Intractable Diseases at Juntendo University Graduate School of Medicine and Lecturer Yohei Honkura from the Department of Otolaryngology and Head and Neck Surgery at Tohoku University Hospital.