

NeuExcell Therapeutics Initiates First-in-Human Clinical Trial of NXL-001 to Treat Alzheimer's Disease Through Neural Regenerative Gene Therapy

Hefei, China, March 5, 2025 — NeuExcell Therapeutics (神曦生物), a biotechnology company at the forefront of neural regeneration gene therapy, announced the launch of a first-in-human clinical trial for Alzheimer's disease (AD) using its proprietary NXL-001. The trial will be conducted at the First Affiliated Hospital of the University of Science and Technology of China (Anhui Provincial Hospital), led by Prof. Jiong Shi in the Department of Neurology as the Principal Investigator, with key support from Prof. Ruobing Qian and Prof. Yinbao Qi in the Department of Neurosurgery.



A Ground-Breaking Approach to Treat Alzheimer's Disease

This investigator-initiated trial (IIT) marks a significant milestone as the world's first clinical study using *in situ* neural regeneration to treat Alzheimer's disease. The trial aims to evaluate the safety/tolerability of NXL-001 in mid- to late-stage AD patients and stop or reverse their disease progression, offering a potential paradigm shift in treating this debilitating disorder.

Alzheimer's disease is the most prevalent neurodegenerative disorder and a leading cause of disability and mortality among the elderly. Current treatments mainly provide

symptomatic relief, and slow down or delay disease progression in early-stage patients.

NXL-001, an AAV-based gene therapy developed by NeuExcell Therapeutics, is designed to directly reprogram internal astrocytes into functional new neurons in the brain. Preclinical studies have demonstrated that NXL-001 can:

- Induce *in situ* neuronal regeneration in both rodents and non-human primates
- Integrate newly generated neurons into existing neural circuits
- Improve cognitive functions in Alzheimer's disease models

"The irreversible loss of neurons is the fundamental pathology of Alzheimer's disease, yet no existing therapies can address this important issue," said Prof. Jiong Shi, the Principal Investigator. "NXL-001 represents a transformative approach by regenerating functional new neurons directly inside the brain, providing a novel therapeutic strategy to potentially redefine AD treatment."

After initiating a glioblastoma trial in 2024, this AD clinical trial marks another important step for NeuExcell in the translation of the cutting-edge gene therapy research into clinic. "Since our 2013 breakthrough study published in *Cell Stem Cell*, which first demonstrated *in situ* neural regeneration in an Alzheimer's disease mouse model, our team has continued to advance this technology to achieve widespread neuronal regeneration and functional recovery," said Prof. Gong Chen, Co-founder and Chairman of NeuExcell Therapeutics. "Initiation of this first-in-human trial represents a significant milestone in our mission to develop innovative therapies to treat millions of patients world-wide suffering from Alzheimer's disease and other neurodegenerative diseases."

About NeuExcell Therapeutics

NeuExcell Therapeutics is a clinical-stage biotechnology company dedicated to developing next-generation therapies to treat neural injuries and neurodegenerative diseases through its proprietary *in situ* neural regeneration platform ATN ASTROCYTES TO NEURONS™ technology. By leveraging this cutting-edge technology, NeuExcell aims to provide transformative solutions for the treatment of neurological disorders such as Alzheimer's disease, stroke, and glioma.

For more information, please visit www.neuexcell.com.