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Australian scientists grow brain tissue in lab to treat rare diseases

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The project will test gene therapies for the first time on patient-derived brain organoids



Brain tissue grown in a laboratory by University of Queensland (UQ) researchers in Australia will be used to test a treatment for a rare disease in children and help unlock therapies for a range of neurological disorders.

Professor Ernst Wolvetang and his team at UQ's Australian Institute for Bioengineering and Nanotechnology (AIBN) have secured almost $1 million from the Medical Research Future Fund (MRFF) to test gene therapies for children with a specific type of Hereditary Spastic Paraplegia (HSP).

HSP Type 56 (SPG56) is a degenerative brain disease that causes children to lose the ability to sit, stand, walk, or talk and there is currently no cure or treatment.

Professor Wolvetang hopes to find one, by testing gene therapies for the first time on patient-derived organoids - tiny, synthetic organs grown from a patient's own cells.

The SPG56 project will be the first time in Australia that brain organoids have been used to test the safety and efficacy of a gene therapy approach for HSP.

Professor Wolvetang said it may help encourage change in the regulatory approval process, which requires extensive testing in animals.

"We hope pre-clinical testing of the efficacy and safety of our methods in patient-specific brain organoids will enable more rapid progress towards human trials," he said.