Center for Gene & Cell Therapy
遺伝子・細胞治療センター

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IMSUT hospital has been playing a lead role in gene therapy and hematopoietic stem cell transplantation in Japan. In order to strengthen this clinical development even further, IMSUT established the Center for Gene & Cell Therapy (CGCT) in 2014. CGCT particularly focuses on the development of gene therapy and cell therapy for intractable cancer and chronic diseases such as oncolytic virus therapy, engineered T cell therapy, gene therapy for neurological disorders using AAV vectors, T cell therapy for post-transplant viral infections, and cell therapy using mesenchymal stem cells.

1. Gene therapy for amyotrophic lateral sclerosis

Shinichi Muramatsu, Sumimasa Nagai and Keiya Ozawa

We developed AAV vectors that can cross the blood- or meningeal-brain barriers. Intra-thecal injections of the AAV vectors will provide a novel platform for treating neurodegenerative diseases, especially where global transduction of a therapeutic gene into the brain is necessary. In sporadic amyotrophic lateral sclerosis (ALS) patients, down regulation of the RNA-editing enzyme, adenosine deaminase, which acts on RNA 2 (ADAR2) is death-causing molecular abnormality that occurs in motor neurons. Gene delivery of the ADAR2 using our new AAV vectors in conditional ADAR2 knockout mice (a mechanistic mouse model of sporadic ALS) effectively prevented progressive motor

dysfunction without any adverse effects. We are going to produce GMP grade AAV vectors that express ADAR2 using baculovirus system for a clinical trial.

Publications

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