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, gene-modified T cell therapy, gene therapy for neurological disorders using AAV vectors, T cell therapy for post-transplant viral infections, and cell therapy using mesenchymal stromal cells.

1. Gene therapy for amyotrophic lateral sclerosis and spinocerebellar ataxia type 6

Shin-ichi Muramatsu, Sumimasa Nagai and Keiya Ozawa

In sporadic amyotrophic lateral sclerosis (ALS) patients, down regulation of the RNA-editing enzyme, adenosine deaminase acting on RNA 2 (ADAR2), is death-causing molecular abnormality that occurs in motor neurons. Gene delivery of the ADAR2 using adeno-associated virus (AAV) vectors in conditional ADAR2 knockout mice effectively prevented progressive motor dysfunction without any adverse effects. We have started to produce GMP grade AAV vectors that express ADAR2 for a clinical trial. In collaboration with the University of Chicago, we have developed miR-based gene therapy for spinocerebellar ataxia type 6 (SCA6). SCA6 is caused by abnormal expansions of the polyglutamine tract within a second *CACNA1A* gene product, α1ACT. Selective translational block of SCA6-

associated α1ACT by delivering miR-3191-5p protected from the Purkinje cell degeneration and ataxia in a mouse model.

Publications

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