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. IMSUT-CGCT Kickoff Symposium 2014

IMSUT-CGCT Kickoff Symposium 2014 was held on Nov. 21th, 2014. More than 200 people from academia, industries and regulatory agencies participated in this symposium. In this symposium, there were lively discussions regarding the following topics; history of gene therapy in Japan, gene therapy for neurodenegerative disorders, oncolytic virus therapy, immunotherapy with CD19-directed chimeric antigen receptor (CAR)-modified T cells (CAR-T cells) for adult patients with relapsed and refractory B cell lymphoma, T cell receptor (TCR) gene-modified T cell therapy for solid tumors, T cell therapy for post-transplant viral infections, cell therapy using mesenchymal stem cells for acute graft-versus-host disease, and regulatory guidelines regarding gene therapy in Japan.

2. Gene therapy for neurodegenerative disorders

Shinichi Muramatsu, Sumimasa Nagai, and Keiya Ozawa

With the increasing life expectancy, the number of patients suffering from neurodegenerative diseases, including Alzheimer's disease (AD), Parkinson's disease (PD) and amyotrophic lateral sclerosis (ALS) will continue to grow. We recently developed new AAV vectors that can cross the blood- or meningeal-brain barriers. We demonstrated robust transductions of neurons and beneficial behavioral effects in model mice of ALS or AD by systemic delivery of these AAV vectors. 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.

Publications

1. Ito, H., Shiwaku, H., Yoshida, C., Homma, H., Luo, H., Chen, X., Fujita, K., Musante, L., Fischer, U., Frints, SGM., Romano, C., Ikeuchi, Y., Shimamura, T., Imoto, S., Miyano, S., Muramatsu, S., Kawauchi, T., Hoshino, M., Sudol, M., Arumughan, A., Wanker, EE., Richi, T., Schwartz, C., Matsuzaki, F., Bonni, A., Kalscheuer, VM. and Okazawa, H. In utero gene therapy rescues microcephaly caused by Pqbp1-hypofunction in neural stem progenitor cells. Mol. Psychiatry. (in press)
2. Miyamoto, Y., Iida, A., Sato, K., Muramatsu, S. and Nitta, A. Knockdown of dopamine D2 receptors in the nucleus accumbens core suppresses methamphetamine-induced behaviors and signal transduction in mice. Int. JNP. (in press)
3. Ito, H., Fujita, K., Tagawa, K., Chen, X., Homma, H., Sasabe, T., Shimizu, J., Shimizu, S., Tamura, T., Muramatsu, S. and Okazawa, H. HMGB1 facilitates repair of mitochondrial DNA damage and extends the lifespan of mutant ataxin-1 knock-in mice. EMBO. Mol. Med. 7: 78-101, 2014.