Press Release

Gene Therapy Research Institution and Astellas Executes Option Agreement for Sporadic ALS Gene Therapy Program

TOKYO, September 14, 2018 –Gene Therapy Research Institution Co., Ltd. (head office: Kawasaki, Kanagawa; President: Katsuhito Asai, "GTRI") and Astellas Pharma Inc. (TSE: 4503, head office: Tokyo, President and CEO: Kenji Yasukawa, Ph.D., "Astellas") today announced that they have entered into an option agreement for the exclusive negotiation of rights to global development and commercialization of the gene therapy program GT0001X for the treatment of sporadic amyotrophic lateral sclerosis (ALS)[1].

ALS is a neurodegenerative disorder that selectively impairs motor neurons, gradually leading to a decrease and loss in strength of muscles in the limbs, throat, tongue, and those required for respiration. There are an estimated 55,000 patients with ALS in Japan, the United States, and the EU5 countries (United Kingdom, Germany, France, Spain, and Italy). The prevalence rate in patients ≥ 60 years of age is as many as 20 to 30 per 100,000 people. The cause of ALS is unknown and no effective therapy to stop the progression of this disease has been established. In patients with sporadic ALS, which accounts for ≥ 90% of the whole ALS population, decreased activity of adenosine deaminase acting on RNA 2 (ADAR2)[2], an RNA-editing enzyme, has been reported to be a possible cause of the disease.

GT0001X is a modified adeno-associated virus (mAAV) vector expressing human ADAR2. In conditional ADAR2 knockout mice, degeneration of anterior horn cells and the resulting motor dysfunction were prevented by mAAV-mediated delivery of ADAR2[3]. Currently, GTRI has been developing GT0001X in the pre-clinical stage for the treatment of sporadic ALS. In the future, the clinical efficacy and safety will be investigated.

"Gene therapy using AAV vectors is about to enter the era of full-scale development in all over the world," said Shin-ichi Muramatsu, MD, Ph.D., Chief Scientific Officer of GTRI. "This gene therapy is highly safe, and widely applicable especially in the field of neurodegenerative diseases and congenital metabolic diseases. GTRI is developing it for many indications. We will conduct clinical trials for sporadic ALS, which is a serious neurodegenerative disease currently without definitive treatment, as our first step. Taking this opportunity, I would like to accelerate development of other programs as well for patients suffering from intractable diseases."

"This agreement demonstrates Astellas' commitment to actively looking to turn state- of-the-art scientific and technological advances into VALUE for patients," said Naoki Okamura, Chief Strategy Officer, Astellas. "Astellas is continuously making efforts to develop medical solutions and new drugs in focused therapeutic areas with high unmet medical needs, an approach that includes collaborating with external partners

performing cutting-edge science. With this agreement, we endeavor to potentially deliver a novel treatment for patients suffering from ALS."

(1) Sporadic amyotrophic lateral sclerosis: There are familial ALS in which ALS patients exist in the blood relatives. It is said that the familial ALS is about 10% of the ALS patient, and the remaining 90% is sporadic ALS

(2) ADAR2 (Adenosine deaminases acting on RNA2): ADAR2 is an RNA-editing enzyme that binds double-stranded RNA and converts adenosine to inosine

(3) EMBO Mol Med. 2013 Nov, vol. 5, no.11, p. 1710-1719.

About GTRI

Gene Therapy Research Institution Co., Ltd., based in Kawasaki, Japan, is a biotech company dedicated to development of AAV vector-mediated gene therapy for intractable diseases. For more information, please visit our website at http://www.en.genetherapy-ri.com/

About Astellas

Astellas Pharma Inc., based in Tokyo, Japan, is a company dedicated to improving the health of people around the world through the provision of innovative and reliable pharmaceutical products. For more information, please visit our website at https://www.astellas.com/en

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