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LSII initiates an exploratory clinical trial to evaluate Muse cell-based product CL2020 for the treatment of amyotrophic lateral sclerosis

Life Science Institute, Inc.

Life Science Institute, Inc. (Headquarters: Chiyoda-ku, Tokyo, President: Seiichi Kiso, hereinafter "LSII") is advancing research and development of products that contribute to the health and well-being of society, such as Muse (multilineage-differentiating stress-enduring) cells for regenerative medicine. At this time, we are very pleased to announce that we are initiating an exploratory clinical trial of the Muse cell-based product CL2020 for the treatment of amyotrophic lateral sclerosis (ALS) in Japan.

ALS is an intractable disease of unknown etiology that mainly develops after middle age and causes the degenerative loss of motor neurons, eventually leading to the loss of control of vital functions such as swallowing and breathing, resulting in death. Currently, ALS has no effective treatment, making it a disease with extremely high unmet medical needs that requires the development of new, effective treatments. Experiments using ALS model mice conducted at the Department of Neurology, Graduate School of Medicine, Dentistry and Pharmaceutical Sciences, Okayama University, demonstrated clear therapeutic effects of intravenous administration of human Muse cells to suppress motor nerve cell loss and motor function decline (Yamashita et al., Sci. Rep., 2020 10(1):17102). Thus, Muse cell-based product CL2020 is also expected to activate and repair nerve cells, making it a potential new treatment for ALS.
Seiichi Kiso, President and CEO of LSII, recently said, "Based on the results of clinical trials conducted so far using CL2020 for other diseases and the results of experiments in ALS model mice conducted in collaboration with Professor Koji Abe of Okayama University, we will be initiating an exploratory clinical trial of ALS earlier than anticipated. Responding to unmet medical needs is the mission of LSII, and our aim is to develop new treatments that will enhance the quality of life of patients with intractable diseases."

The use of CL2020 in clinical trials for ALS is the sixth proposed indication of

CL2020 for treating human disease, following: acute myocardial infarction, cerebral infarction, epidermolysis bullosa, spinal cord injury, and neonatal hypoxic-ischemic encephalopathy (investigator-initiated clinical trial).
The LSII Group is committed to improving the health and medical care of people around the world by developing healthcare businesses and products that will contribute to the realization of a healthy and secure society, KAITEKI, for future generations.

Muse cells
Muse cells are a novel type of pluripotent repair stem cell discovered by Professor Mari Dezawa's group at Tohoku University in 2010. Muse cells are endogenous to the human body and exist in the peripheral blood, bone marrow, and connective tissue of each organ. Due to their unique characteristics, Muse cells are expected to play an integral role in numerous regenerative therapies for a wide range of diseases. Systemically administered donor-derived Muse cells can spontaneously differentiate into tissue-compatible cells in the body without the need for prior complex processing. Simple intravenous injection delivers Muse cells to damaged tissue where they selectively accumulate to the damaged tissue, and engraft to repair the tissue by cell replacement. Muse cells also exert an immunomodulatory function, and thus donor-derived Muse cells remain immune-privilege without requiring donor-recipient matching or immunosuppressants. As Muse cells are endogenous, they have few safety concerns and low tumorigenicity, making them an ideal substrate for natural regenerative products.

Amyotrophic Lateral Sclerosis (ALS)
ALS is a disease of unknown etiology in which the muscles of the limbs, throat, tongue, and respiratory system gradually deteriorate and lose strength, and has been designated an intractable disease by the Japanese government. In ALS, the problem does not originate with the muscles themselves, but rather with degeneration of the upper motor neurons that make up the corticospinal tract, which transmits commands from the brain to the muscles, and the lower motor neurons that control the skeletal muscles by instructions from the brain. The signals from the brain are not transmitted to the muscles, leading to the gradual weakening of muscle strength and eventual muscle deterioration (motor dysfunction). ALS is a progressive illness with widespread systemic effects and persistent symptoms. In Japan, approximately 9,800 people have ALS. No effective treatment is currently available, and thus the development of new treatments is crucial.