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Summary Results of a Clinical Trial with Muse cell-based product, CL2020, in Patients with Epidermolysis Bullosa

Life Science Institute, Inc.

Life Science Institute, Inc. or LSII (Head office: Chiyoda-ku, Tokyo; President: Seiichi Kiso) is pleased to announce summary results of a clinical trial with Muse cell-based product, CL2020, in patients with epidermolysis bullosa, which has been conducted at HOKKAIDO UNIVERSITY HOSPITAL, Toho University Omori Medical Center and other clinical sites in Japan since December 2018.

CL2020 successfully demonstrated its favorable safety profiles up to 52 weeks after a single-dose intravenous administration according to the clinical trial report. The efficacy endpoint of this trial was also achieved. More details will be presented at academic conferences or will be published in scientific journals in the future. LSII will continue the clinical development of CL2020 for this indication in consultation with regulatory authorities.

LSII is committed to contribute to people's health and well-being around the world by developing the next-generation technologies, including Muse cell-based product and to creation of a society where everyone can live a healthy and peaceful life, KAITEKI.

About "Muse cells"

Muse cells (multilineage-differentiating stress enduring cells), discovered by Professor Mari Dezawa's group at Tohoku University in 2010, are a novel type of non-tumorigenic pluripotent stem cells that can be differentiated into various kinds of cells in the body. Muse cells are endogenous reparative stem cells distributed in the peripheral blood, bone marrow and connective tissue of organs. Their advantageous characteristics are represented by low safety concerns, unnecessary of gene introduction or differentiation induction prior to administration and of surgical operation for delivering cells because of their specific ability to accumulate to the damaged site after intravenous administration, enabling treating patients only by intravenous drip of Muse cell preparation, one of the simple expedient approaches.

About "Epidermolysis Bullosa"

Epidermolysis bullosa is a disease where the skin develops blisters and ulcers caused by rubbing of the skin and mucous membranes. The disease results from the inability to withstand force applied to the skin in the course of daily life due to the shortage or lack of adhesion structure molecules responsible for the epidermis-basement membrane-dermis adhesion. It is a designated intractable disease with an estimated 500 to 1,000 patients in Japan. Because there is no effective therapy at present, the development of a new treatment method is desired.