

**Results of a Clinical Trial  
with Muse cell-based product, CL2020,  
in Patients with Epidermolysis Bullosa  
Published in *the Journal of the European Academy of  
Dermatology and Venereology Online***

Life Science Institute, Inc.

Life Science Institute, Inc. (Headquarters: Chiyoda-ku, Tokyo, President: Seiichi Kiso, hereinafter "LSII") is pleased to announce that results from a phase 1/2 open label clinical trial of Muse cell-based product, CL2020 for epidermolysis bullosa was published on March 14, 2021 (local time) in the online version of *the Journal of the European Academy of Dermatology and Venereology*. LSII announced in July 2020 the summary results of the study, which had been conducted at HOKKAIDO UNIVERSITY HOSPITAL, Toho University Omori Medical Center and other clinical sites in Japan since 2018.

This study was conducted to evaluate safety and efficacy of a single intravenous dose of CL2020 in patients with refractory skin ulcers in epidermolysis bullosa (5 patients). As the result, CL2020 demonstrated its favorable safety profiles up to 52 weeks after administration as the primary endpoint. Regarding efficacy as a secondary endpoint, the significant improvement in change of the total size of ulcers was observed at 4 weeks after CL2020 treatment.

More details are available at <https://onlinelibrary.wiley.com/doi/10.1111/jdv.17201>

LSII expects that CL2020 can be a promising treatment option for patients with severe epidermolysis bullosa, as CL2020 suggested its favorable safety and tolerability in this study. Based on these results, 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.

### **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.

### **Epidermolysis Bullosa**

Epidermolysis bullosa (EB) is a disease where the skin develops blisters and ulcers caused by rubbing of the skin and mucous membranes. In some cases, blisters form inside the body in places such as the mouth, esophagus, other internal organs, or eyes. 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. EB is one of designated intractable diseases with an estimated 500 to 1,000 patients in Japan. Development of a new treatment option that can modify the disease is expected because there is currently no effective therapy.