

## **StemRIM Announces Publication of Scientific Articles for Stem Cell Gene Therapy (PJ5)**

**Osaka, Japan, May 16, 2023** – StemRIM Inc. (TSE: 4599, Chairman and CEO: Kensuke Tomita; “StemRIM”) announced that a research paper summarizing the results of our collaborative research on the development of stem cell gene therapy for epidermolysis bullosa (Development code; PJ5), conducted in partnership with Osaka University, has been published in the *Journal of Investigative Dermatology* (JID), the official journal of the Society for Investigative Dermatology in the United States.

The title of this research paper is "Gene-modified blister fluid-derived mesenchymal stromal cells for treating recessive dystrophic epidermolysis bullosa". Based on the development of a novel technique for isolating and culturing mesenchymal stem cells from blister fluid of patients with severe recessive dystrophic epidermolysis bullosa, who have a deficiency of type VII collagen, the study reports on the introduction of the type VII collagen gene into patient-derived mesenchymal stromal cells from blister fluid and subsequent transplantation of these cells back into the patient's skin. This approach aims to achieve a curative treatment for recessive dystrophic epidermolysis bullosa, a condition for which there is currently no effective therapy available. This research achievement represents an important step toward the practical application of stem cell gene therapy, and it is expected to accelerate further research and development toward a curative treatment for recessive dystrophic epidermolysis bullosa.

Furthermore, as announced in the "Notice of Participation as a Collaborative Research Company in the AMED Public Offering Project 'FY 2022 Research for Practical Application of Intractable Diseases'" dated April 5, 2022, in Japanese ([140120220404517390.pdf \(xj-storage.jp\)](#)), our joint research partner (Osaka University) has received funding for the clinical trial expenses from the Japan Agency for Medical Research and Development (AMED). Currently, preparations for the clinical trial are underway at Osaka University.

### **About StemRIM Inc.**

StemRIM Inc. is a biotech venture which began at Osaka University with the goal of realizing a new type of medicine called "Regeneration-Inducing Medicine™". The overall aim is to achieve regenerative therapy effects equivalent to those of regenerative medicine, solely through drug administration, without using living cells or tissues. Living organisms have inherent self-organizing abilities to repair and regenerate tissues that have been damaged or lost due to injury or disease. This ability arises from the presence of stem cells in the body that exhibit pluripotency i.e., can differentiate into various types of tissues. When tissues are damaged, these cells, therefore, exhibit proliferative and differentiative capabilities, promoting functional tissue regeneration. "Regeneration-Inducing Medicine™" is aimed at maximizing the tissue repair and regeneration mechanisms already present in the body. With this aim, StemRIM is currently developing one of its most advanced regenerative medicine products. Specifically, this product is designed to release (mobilize) mesenchymal stem cells

from the bone marrow into the peripheral circulation upon administration, thus increasing the number of stem cells circulating throughout the body and promoting their accumulation in damaged tissues. Here, these stem cells should accelerate tissue repair and regeneration. Certain disease areas expected to benefit from "Regeneration-Inducing Medicine™" include epidermolysis bullosa (EB), acute phase cerebral infarction, cardiomyopathy, osteoarthritis of the knees, chronic liver disease, myocardial infarction, pulmonary fibrosis, traumatic brain injury, spinal cord injury, atopic dermatitis, cerebrovascular disease, intractable skin ulcers, amyotrophic lateral sclerosis (ALS), ulcerative colitis, non-alcoholic steatohepatitis (NASH), systemic sclerosis, and any other areas where treatment with extrapulmonary mesenchymal stem cells is promising.

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