May 24, 2023
StemRIM Inc.

StemRIM Announces Orphan Drug Designation of Regeneration-Inducing Medicine™ Redasemtide (HMGB1 peptide)

Osaka, Japan, May 24, 2023 – StemRIM Inc. (TSE: 4599, Chairman and CEO: Kensuke Tomita; "StemRIM") announced, that Regeneration- Inducing Medicine ™ Redasemtide (HMGB1 peptide) licensed to Shionogi & Co., Ltd. (TSE: 4507, Chief Executive Officer: Isao Teshirogi, Ph.D.; "Shionogi") was designated as an orphan drug for the treatment of Dystrophic epidermolysis bullosa by the Ministry of Health, Labour and Welfare ("MHLW").

The designation system for orphan drugs, orphan medical devices, and products for regenerative medicine for rare diseases is a support measure to promote research and development on orphan drugs, etc. Based on the designated criteria below, MHLW designates these products after receiving advice from the Pharmaceutical Affairs and Food Sanitation Council.

1. The number of patients who may use the drug or product should be less than 50,000 in Japan.
2. The drugs or products should be indicated for the treatment of serious diseases. In addition, they must be drugs or products for which there are high medical needs satisfying one of the following criteria.
   - There is no appropriate alternative drug/medical device or treatment.
   - High efficacy or safety is expected compared with existing products.
3. There should be a theoretical rationale for the use of the drug or product for the target disease, and the development plan should be considered appropriate.

The recent designation of Redasemtide as an orphan drug indicated that it has received a certain level of recognition and evaluation from MHLW regarding its potential effectiveness against epidermolysis bullosa and the validity of its current development plan.

By receiving the designation as an orphan drug, Shionogi can take some incentives from the following support measures:

1. Subsidy payment: Orphan drug/medical device applicants can receive subsidies through the National Institute of Biomedical Innovation (NIBIO) to reduce the financial burden of product development.
2. Guidance and consultation: Orphan drugs/products can receive guidance and consultation from the Pharmaceuticals and Medical Devices Agency (PMDA) and other relevant authorities. PMDA provides a priority consultation system for designated orphan drug/medical device. Lower user fee categories for PMDA's consultation are applicable to designated orphan drugs.
3. Preferential tax treatment: 20% of study expenses for orphan drug/product incurred during the NIBIO subsidy payment period (not including subsidies granted by NIBIO) can be reported as a tax credit.
4. Priority review: Designated orphan drugs will be subject to priority review for marketing authorization to ensure that they are supplied to clinical settings at the earliest possible opportunity. Categories of lower user fees are applicable to review for marketing authorization of designated orphan drugs.

5. Extension of re-examination period: After orphan drug/medical device designation and approval, the re-examination period for the drugs will be extended up to 10 years for drugs During this period, no applications for non-new drugs are accepted, allowing for substantial exclusive sales even beyond the patent expiration.

The priority review system is a system implemented in Japan that prioritizes the review of certain drugs that meet specific criteria during the approval process for new drugs. This system was introduced in Japan in 1993 through partial revisions of the Pharmaceutical Affairs Law (now known as the Pharmaceuticals and Medical Devices Act). Priority review for pharmaceuticals in Japan is granted to new drugs and other products that are recognized to have high medical utility for serious diseases.

By being eligible for priority review, it is expected to expedite the approval process and enable an earlier market launch.

This matter is progressing as planned and has no impact on our financial results for the fiscal year ending July 31, 2023.

About StemRIM Inc.

StemRIM Inc. is a biotech venture which began at Osaka University to realize 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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For more information, please visit the StemRIM website (https://stemrim.com/english/)