PRESSRELEASE

SHIONOGI

Orphan Drug Designation for Redasemtide for Dystrophic Epidermolysis Bullosa

OSAKA, Japan, May 24, 2023 - Shionogi & Co., Ltd. (Head Office: Osaka, Japan; Chief Executive Officer: Isao Teshirogi, Ph.D.; hereafter "Shionogi") announced that redasemtide, a regeneration inducing medicine® in-licensed from StemRIM Inc. (Headquarters: Ibaraki City, Osaka; Chairman and CEO: Kensuke Tomita; hereafter "StemRIM"), was designated as an orphan drug for dystrophic epidermolysis bullosa by the Ministry of Health, Labor and Welfare (MHLW).

Orphan drugs are drugs designated by MHLW after consultation by the Pharmaceutical Affairs and Food Sanitation Council based on prescribed standards[1]. With this designation, early approval and launch of redasemtide are expected by shortening the review period by authorities.

Shionogi is conducting an additional Phase 2 clinical trial[2] in patients with dystrophic epidermolysis bullosa. This is a multicenter, open-label, uncontrolled trial designed to evaluate the efficacy for intractable ulcers of redasemtide. Target number of subjects is 3 or more, and first enrollment was completed in February 2023. We continue to drive to provide redasemtide to patients as soon as possible.

About redasemtide

Redasemtide is a regeneration-inducing medicine® under development that regenerates tissues damaged by injury or disease without using living cells. In order to provide healthcare solutions to as many patients as possible by taking advantage of the characteristics of regeneration-inducing medicine®, we are engaging in its development for acute ischemic stroke, chronic liver disease, knee osteoarthritis, and cardiomyopathy, in addition to dystrophic epidermolysis bullosa.

About dystrophic epidermolysis bullosa[3]

Epidermolysis bullosa (EB) is an inherited, heterogeneous group of rare genetic dermatoses characterized by mucocutaneous fragility and blister formation, inducible by often minimal trauma. A broad phenotypic spectrum has been described, with potentially severe extracutaneous manifestations, morbidity and mortality. Over 30 subtypes are recognized, grouped into four major categories, based predominantly on the plane of cleavage within the skin and reflecting the underlying molecular abnormality: EB simplex, junctional EB, dystrophic EB and Kindler EB. The number of EB patients in Japan is estimated to be approximately 500 to 1,000, but there is currently no radical treatment.

About StemRIM

StemRIM is a drug discovery research and development oriented biotech company originating from Osaka University. It was established in 2006 with the aim of developing a myelomultiactive stem cell recruitment factor as a pharmaceutical product, which was identified by Professor Tamai and his colleagues at the Graduate School of Medicine, Osaka University. Since then, through joint research with Osaka University, StemRIM has been consistently pursuing the development of “regeneration-inducing medicine”, which is medicine promoting functional tissue regeneration and enabling the treatment of previously intractable

diseases. StemRIM is continuing to undertake the challenge of becoming a world-leading bioventure company with the corporate mission of "overcoming intractable diseases with regeneration-inducing medicine." For more information, please refer to the StemRIM website.

Forward-Looking Statements

This announcement contains forward-looking statements. These statements are based on expectations in light of the information currently available, assumptions that are subject to risks and uncertainties which could cause actual results to differ materially from these statements. Risks and uncertainties include general domestic and international economic conditions such as general industry and market conditions, and changes of interest rate and currency exchange rate. These risks and uncertainties particularly apply with respect to product-related forward-looking statements. Product risks and uncertainties include, but are not limited to, completion and discontinuation of clinical trials; obtaining regulatory approvals; claims and concerns about product safety and efficacy; technological advances; adverse outcome of important litigation; domestic and foreign healthcare reforms and changes of laws and regulations. Also for existing products, there are manufacturing and marketing risks, which include, but are not limited to, inability to build production capacity to meet demand, lack of availability of raw materials and entry of competitive products. The company disclaims any intention or obligation to update or revise any forward-looking statements whether as a result of new information, future events or otherwise.

Reference

1. Orphan Drug Designation
   This is a supportive measure to promote research and development of orphan drugs.
   MHLW designate after consultation by the Pharmaceutical Affairs and Food Sanitation Council based on prescribed standards such as that the number of target patients is less than 50,000 in Japan, that the target disease is a serious disease with a particularly high medical need. For more information, please refer to the MHLW website.
2. Japan Registry Clinical Trials
3. https://www.nature.com/articles/s41572-020-0210-0

For Further Information, Contact:

SHIONOGI Website Inquiry Form: https://www.shionogi.com/global/en/contact.html