



## **Shionogi Announces First Patients Enrolled in Global Phase 2 Clinical Trial in Adults with Late-Onset Pompe Disease**

*S-606001 has the potential to become the first oral substrate reduction therapy for Pompe disease*

**OSAKA, Japan, March 19, 2026** – Shionogi & Co., Ltd. (Head Office: Osaka, Japan; Chief Executive Officer: Isao Teshirogi, Ph.D.; hereafter “Shionogi”) announced the first patients were enrolled in Esprit, a global Phase 2 clinical trial evaluating S-606001, an investigational drug for the treatment of late-onset Pompe disease (LOPD).

Esprit is a multicenter, randomized, placebo-controlled, double-blind study evaluating the safety, pharmacodynamics and preliminary efficacy of S-606001 as an oral substrate reduction therapy (SRT) in addition to standard of care enzyme replacement therapy (ERT) in adults with a confirmed diagnosis of LOPD.<sup>1</sup> This 52-week study will enroll participants across the U.S., European Union and United Kingdom.<sup>1</sup>

Pompe disease is a rare genetic metabolic disorder that presents in children and adults.<sup>2,3</sup> In people with Pompe disease, a deficiency of the acid alpha-glucosidase (GAA), an enzyme necessary for the breakdown of glycogen, results in the accumulation of glycogen in tissues throughout the body, especially in muscles.<sup>3</sup> In LOPD, GAA activity is partially reduced.<sup>3</sup> This can cause severe weakness and respiratory issues leading to respiratory insufficiency, wheelchair dependency and a shortened lifespan.<sup>4</sup> LOPD affects about one in every 22,000 people worldwide and may be identified at any age.<sup>5,6</sup> Despite significant progress in diagnosis in countries with newborn screening programs, identifying LOPD in people who are not screened at birth remains challenging.<sup>5</sup> Its rarity, wide range of clinical presentations, and overlap with other neuromuscular disorders often lead to delays in diagnosis.<sup>7,8</sup>

S-606001 is an investigational SRT that is believed to work by limiting glycogen buildup in muscle lysosome by inhibiting glycogen synthase (GYS1).<sup>1,9</sup> ERT, the current approved treatment for Pompe disease, infuses more GAA enzyme to increase glycogen breakdown.<sup>10</sup> SRT blocks the GYS1 enzyme to slow down glycogen buildup.<sup>9,11</sup> Because SRT targets the opposite side of the

glycogen buildup problem from ERT, it has the potential to work alone or in combination with ERT.<sup>9,11</sup>

“The Pompe community is greatly appreciative of Shionogi’s commitment to developing new treatment options for people living with late-onset Pompe disease. Each person deserves alternatives to help them best manage their condition,” said Brad Crittenden, Chairman, International Pompe Association and Executive Director, Canadian Association of Pompe.

“Currently, ERTs are the standard of care for LOPD, but their efficacy can wane over time, leading to continued decline in skeletal muscle function. There is a significant unmet need for new treatment approaches that can be complementary to existing treatments to further slow disease progression,” said Juan Carlos Gomez, M.D., Chief Medical Officer, Shionogi & Co., Ltd. “This is an important milestone for Shionogi, as we continue to expand our work in rare disease, and we hope it will prove to be an important step forward for the Pompe community.”

Shionogi acquired exclusive worldwide rights for S-606001 (previously known as MZE001) from Maze Therapeutics, Inc. in 2024. In 2025, S-606001 received a rare pediatric disease designation from the U.S. Food and Drug Administration (FDA) for the treatment of Pompe disease, a designation granted for serious and life-threatening diseases that primarily affect children ages 18 years or younger with fewer than 200,000 people in the U.S. The FDA also granted Orphan Drug Designation to the compound in 2022.

Additional details about Esprit are available at [ClinicalTrials.gov ID: NCT07123155](https://clinicaltrials.gov/ct2/show/study/NCT07123155) and on [www.espritstudy.com](http://www.espritstudy.com).

S-606001 is currently under investigation in clinical trials for the treatment of late-onset Pompe disease. The safety and effectiveness of S-606001 have not been established, nor has it been approved by FDA or any health authority.

### **About Shionogi in Rare Disease**

Shionogi is committed to the research and development of innovative medicines that address unmet medical needs for people worldwide. Rare diseases affect individuals and families around the world and treatment options for rare diseases are often limited. Shionogi is advancing clinical programs for rare diseases and disorders including Fragile X syndrome, Jordan’s Syndrome and Pompe disease. In December 2025, Shionogi announced plans to expand its rare disease portfolio through the acquisition of all global rights, including in Japan and the United States, to the treatment for amyotrophic lateral sclerosis (ALS) developed and marketed by Tanabe Pharma Co., Ltd.

## **About Shionogi & Co., Ltd.**

Shionogi & Co., Ltd. is a 148-year-old global, research-driven pharmaceutical company headquartered in Osaka, Japan, that is dedicated to bringing benefits to patients based on its corporate philosophy of “supplying the best possible medicine to protect the health and wellbeing of the patients we serve.” The company currently markets products in several therapeutic areas including anti-infectives, pain, CNS disorders and cardiovascular diseases. Shionogi’s research and development currently targets two therapeutic areas: infectious diseases and diseases with unmet medical needs in pain/CNS, including Alzheimer’s disease, oncology, rare diseases, and sleep apnea. For more information on Shionogi & Co., Ltd., please visit <https://www.shionogi.com/global/en>.

## **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.*

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<sup>1</sup> Study of S-606001 as an Add-on to Enzyme Replacement Therapy (ERT) in Participants with Late-onset Pompe Disease (LOPD). Clinicaltrials.gov. Available at:

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<sup>2</sup> Pompe disease. Boston Children’s Hospital. Available at:

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