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Japan's Shionogi announces $150 M deal to develop first oral therapy for Pompe Disease

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Agreement adds to Shionogi’s growing pipeline in rare disease

Japan-based Shionogi & Co. and Maze Therapeutics, Inc., in the US, have completed an exclusive worldwide license agreement for the rights to MZE001, an investigational oral glycogen synthase 1 (GYS1) inhibitor that aims to address Pompe disease by limiting disease-causing glycogen buildup.

Pompe disease is a rare, inherited disorder caused by mutations in the gene coding for acid alpha-glucosidase (GAA), which can lead to the buildup of glycogen in skeletal muscle, respiratory muscle and cardiac muscle tissues resulting in progressive weakness and respiratory compromise.

Under the terms of the agreement, Shionogi has acquired exclusive worldwide rights for MZE001 as well as related programmes and intellectual property. Shionogi will pay an upfront fee of $150 million, and Maze will be eligible for milestone payments based on development, regulatory and commercial achievements plus tiered royalties based upon future net sales.

MZE001 is a small molecule and specific inhibitor of GYS1, an enzyme involved in glycogen synthesis. It reduces the glycogen concentration in muscles by inhibiting this enzyme, and the results from the Phase 1 study of MZE001 suggest that it has the potential to be the first oral therapy for the treatment of Pompe disease. MZE001 has the potential to be used both as a monotherapy option and as an add-on therapy with enzyme replacement, the current standard of care, to enhance the treatment of patients with Pompe disease.

In 2022, the US Food and Drug Administration (FDA) granted Orphan Drug Designation to MZE001.