

Ono Enters into License Agreement with Ionis Pharmaceuticals for Sapablursen for the Treatment of Polycythemia Vera

Osaka, Japan, March 12, 2025 – Ono Pharmaceutical Co., Ltd. (Headquarters: Osaka, Japan; President and COO: Toichi Takino; “ONO”) announced that it has entered into a license agreement with Ionis Pharmaceuticals, Inc. (Carlsbad, CA, U. S.; CEO, Brett P. Monia, Ph.D.; “Ionis”) for sapablursen, an investigational RNA-targeted medicine for the treatment of polycythemia vera (PV). Sapablursen is currently being evaluated in adult patients with PV in the fully enrolled Phase 2 IMPRSSION study. Sapablursen was granted Fast Track designation in January 2024 and orphan drug designation in August 2024 by the U.S. Food and Drug Administration (FDA).

Under the agreement, ONO will obtain an exclusive license to develop and commercialize sapablursen worldwide. Ionis will remain responsible for the completion of the ongoing Phase 2 IMPRSSION study, while ONO will be solely responsible for subsequent development, regulatory filings and commercialization.

ONO will make an upfront payment of 280 million US dollars, with up to a maximum of 660 million US dollars in additional payments based on the achievement of development, regulatory and sales milestones. ONO will also pay to Ionis royalties in the mid-teens on annual net sales of sapablursen. The transaction is subject to the requirements of the Hart-Scott-Rodino Antitrust Improvements Act of 1976 (HSR Act).

“We are delighted to enter into this collaboration with Ionis Pharmaceuticals, a pioneer in RNA-targeted medicines, for sapablursen. This partnership aligns with our strategy to strengthen our pipeline in hematology. We expect sapablursen to become a new treatment option for PV patients worldwide,” said Toichi Takino, President and COO of ONO.

“Ionis and ONO share a commitment to advancing innovative medicines to help meet the needs of people living with serious diseases. We are pleased to entrust sapablursen to ONO, whose unique capabilities will help maximize its value by ensuring broad access for people living with PV,” said Brett P. Monia, Ph.D., CEO of Ionis.

The impact of this matter on the Company’s consolidated financial results for the current fiscal year ending March 31, 2025, is expected to be minor. Should there be any events to be disclosed in the future, we will announce them in a timely manner.

About Sapablursen

Sapablursen is designed to reduce the production of TMPRSS6 resulting in increased expression of hepcidin, which is the key regulator of iron homeostasis. By increasing production of hepcidin, sapablursen has the potential to positively impact blood diseases such as PV.

About Polycythemia Vera

Polycythemia Vera (PV) is a rare and potentially life-threatening hematologic disease characterized by the overproduction of red blood cells, which significantly increases the risk of serious blood clots, especially in critical organs like the lungs, heart and brain. Patients with PV also experience severe iron deficiency and commonly have symptoms of fatigue, which can lead to reduce quality of life (QOL).

About Ionis Pharmaceuticals Inc.

For three decades, Ionis has invented medicines that bring better futures to people with serious diseases. Ionis currently has six marketed medicines and a leading pipeline in neurology, cardiology, and select areas of high patient need. As the pioneer in RNA-targeted medicines, Ionis continues to drive innovation in RNA therapies in addition to advancing new approaches in gene editing. A deep understanding of disease biology and industry-leading technology propels our work, coupled with a passion and urgency to deliver life-changing advances for patients. For further information, visit <https://www.ionis.com/>.

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