



Ionis and Ono announce global license agreement for sapablursen in polycythemia vera

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CARLSBAD, Calif. & OSAKA, Japan--(BUSINESS WIRE)--Mar. 11, 2025-- [Ionis Pharmaceuticals, Inc.](#) (Nasdaq: IONS) and Ono Pharmaceutical Co., Ltd (Ono), today announced that the two companies have entered into a license agreement in which Ono obtains exclusive global rights for the development and commercialization of sapablursen, an investigational RNA-targeted medicine for polycythemia vera (PV), a rare and potentially life-threatening hematologic disease. Sapablursen is currently being evaluated in adults living with PV in the fully enrolled Phase 2 IMPRSSION study. Sapablursen was granted Fast Track designation and orphan drug designation in 2024 by the U.S. Food and Drug Administration (FDA).

Under the terms of the agreement, Ionis will receive a \$280 million upfront payment, with the potential to earn up to \$660 million in additional payments based on the achievement of development, regulatory and sales milestones. Ionis is also eligible to earn royalties in the mid-teen percentage range on annual net sales of sapablursen. Ionis will be responsible for the completion of the ongoing Phase 2 IMPRSSION study, while Ono will be solely responsible for subsequent development, regulatory filings and commercialization. The transaction is subject to the requirements of the Hart-Scott-Rodino Antitrust Improvements Act of 1976 (HSR Act).

“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., chief executive officer, Ionis. “Ionis remains committed to advancing the wholly owned medicines we choose to commercialize ourselves, which includes our first independent launch currently underway and three additional anticipated launches in the next three years. Streamlining our Ionis-owned portfolio provides financial flexibility, supporting our commitment to invest in and focus on our near and mid-term commercial opportunities and generate substantial revenue growth.”

“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, Ono.

About Polycythemia Vera (PV)

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.

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 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. To learn more about Ionis, visit [ionis.com](#) and follow us on [X](#) (Twitter), [LinkedIn](#) and [Instagram](#).

About Ono Pharmaceutical Co., Ltd.

Ono Pharmaceutical Co., Ltd., headquartered in Osaka, Japan, is an R&D-oriented pharmaceutical company dedicated to creating innovative medicines in specific disease areas. Ono Pharma focuses on oncology, immunology, neurology, and specialty research, prioritizing areas with high medical needs for the discovery and development of innovative new treatments. Founded in 1717 in Osaka, the company has facilities in Japan, the UK, the US, South Korea, and Taiwan. In June 2024, Ono Pharma acquired Deciphera Pharmaceuticals Inc., based in Massachusetts, USA, to strengthen its drug pipeline and support global expansion efforts.

For further information visit: <https://www.ono-pharma.com/en>

Ionis Forward-Looking Statements

This press release includes forward-looking statements regarding Ionis' business and the therapeutic and commercial potential of sapablursen, Ionis' technologies and other products in development. Any statement describing Ionis' goals, expectations, financial or other projections, intentions or beliefs is a forward-looking statement and should be considered an at-risk statement. Such statements are subject to certain risks and uncertainties, including but not limited to those related to our commercial products and the medicines in our pipeline, and particularly those inherent in the process of discovering, developing and commercializing medicines that are safe and effective for use as human therapeutics, and in the endeavor of building a business around such medicines. Ionis' forward-looking statements also involve assumptions that, if they never materialize or prove correct, could cause its results to differ materially from those expressed or implied by such forward-looking statements. Although Ionis' forward-looking statements reflect the good faith judgment of its management, these statements are based only on facts and factors currently known by Ionis. Except as required by law, we undertake no obligation to update any forward-looking statements for any reason. As a result, you are cautioned not to rely on these forward-looking statements. These and other risks concerning Ionis' programs are described in additional detail in Ionis' annual report on Form 10-K for the year ended Dec. 31, 2024, and most recent Form 10-Q, which are on file with the SEC. Copies of these and other documents are available at www.ionis.com.

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