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Press Release

Ono Announces Oral Presentation of Positive Topline Results from Phase 2a Study of Sapablursen in Polycythemia Vera at the 67th American Society of Hematology (ASH) Annual Meeting

- Results from Phase 2a IMPRSSION study demonstrate sapablursen significantly reduced phlebotomy rate, controlled hematocrit and increased serum hepcidin
- Sapablursen was generally safe and well tolerated
- Results support further development of sapablursen in a Phase 3 study

Osaka, Japan, December 8, 2025 – Ono Pharmaceutical Co., Ltd. (Headquarters: Osaka, Japan; President and COO: Toichi Takino; "Ono"), today announced the oral presentation of positive results from the Phase 2a IMPRSSION study of sapablursen in patients with polycythemia vera (PV) at the 67th American Society of Hematology (ASH) Annual Meeting, taking place December 6-9, 2025, in Orlando, Florida.

The results were presented by Ionis Pharmaceuticals, who discovered and developed sapablursen and conducted the IMPRSSION study. In March 2025, Ionis and Ono entered into a license agreement in which Ono obtained exclusive global rights for the development and commercialization of sapablursen.

"In the treatment of PV, phlebotomy and cytoreductive therapy are performed as treatments for preventing thrombosis. Phlebotomy is the most common treatment for PV, in which blood is regularly removed from the vein, but it imposes significant physical and psychological burdens on patients. These Phase 2a study results demonstrate the ability of sapablursen to reduce the rate of blood withdrawals and control the hematocrit, which is the percentage of red blood cells in the total blood volume of the body, in phlebotomy-dependent patients including those undergoing cytoreductive therapy," said Tatsuya Okamoto, Corporate Officer / Executive Director, Clinical Development of Ono. "We believe sapablursen has the potential to be an important new treatment option for patients with PV and we look forward to advancing this promising treatment in a Phase 3 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), along with Breakthrough Therapy designation in May 2025. Based on the positive Phase 2a study, Deciphera plans to initiate a Phase 3 study of sapablursen in patients with PV in 2026.

Summary of Data and Findings from Phase 2a IMPRSSION Study

The Phase 2a IMPRSSION study is a multicenter, randomized, open-label trial evaluating the safety and efficacy of sapablursen in patients with phlebotomy-dependent PV. Forty-nine (49) patients were accrued to Cohort A (N=32) and Cohort B (N=17). Cohort A initially assessed 120 mg before the dose was reduced to 80 mg, and Cohort B tested 40 mg. Sapablursen was administered subcutaneously every four weeks. The treatment period was 37 weeks, with an endpoint window between weeks 17 and 37, followed by a 36-week treatment extension period.

Efficacy

- In both cohorts, the study achieved its primary endpoint of significantly decreasing weekly phlebotomy rate from baseline to weeks 17-37; with a decrease from 0.15 to 0.05 in Cohort A ($p<0.0001$) and from 0.17 to 0.07 in Cohort B ($p=0.0001$).
- In patients that completed the 37-week treatment period, the median number of phlebotomies during the last 20 weeks of treatment (weeks 17-37) decreased to 0 and 1.5 phlebotomies in Cohort A and B, respectively, compared to 5 phlebotomies in the 26 weeks (6 months) prior to treatment for both cohorts.
- Sapablursen caused a dose- and time-dependent increase in hepcidin with a corresponding reduction in hematocrit.
- When assessing the symptoms of PV via Myeloproliferative Neoplasm Symptom Assessment Form - Total Symptom Score (MPN-SAF-TSS), the mean change from baseline was statistically significant in Cohort A and not statistically significant for Cohort B.
    - In Cohort A there was a mean change of -6.2.
    - In Cohort B there was a mean change of -2.7.

Safety

- Sapablursen was generally safe and well tolerated.
- During the study, one death occurred due to transformation to acute myeloid leukemia, which was deemed not related to the study drug.
- The incidence of injection site reactions was low.
- Injection site reactions were all mild in severity, not progressive, resolved spontaneously, and did not recur.
- No laboratory trends suggesting adverse effects on liver or renal function were observed.

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).

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