ONO PHARMA

Press Release

Ono Pharma Receives Orphan Medicinal Product Designation for Sapablursen from European Commission and Plans to Present Trial-in-Progress Poster at ASH 2026

- Design of Phase 3 INTREPID study of sapablursen in polycythemia vera to be presented at the 68th American Society of Hematology Annual Meeting
- Sapablursen has also received Orphan Drug Designations for polycythemia vera in the United States, Japan and Europe

Osaka, Japan, November 5, 2026 -- Ono Pharmaceutical Co., Ltd. (Headquarters: Osaka, Japan; President and COO: Toichi Takino; "Ono") today announced that the European Commission has granted Orphan Medicinal Product Designation to sapablursen (ONO-0530) in polycythemia vera (PV). Sapablursen has previously received Fast Track Designation in January 2024, Orphan Drug Designation in August 2024, and Breakthrough Therapy Designation in May 2025 from the U.S. Food and Drug Administration (FDA). In Japan, sapablursen received Orphan Drug Designation in September 2026.

In addition, a trial-in-progress poster about the global Phase 3, double-blind, randomized controlled INTREPID study of sapablursen in patients with PV, which is being developed by Deciphera, Ono's U.S. subsidiary, will be presented at the 68th American Society of Hematology Annual Meeting, being held December 12-15, 2026, in New Orleans, Louisiana.

The poster presentation details are as follows:

Title: Trial in Progress: INTREPID: A global, randomized, double-blind, placebo-controlled Phase 3 study of sapablursen in patients with phlebotomy-dependent polycythemia vera

Presenter: Pankit Vachhani, M.D., Associate Professor of Medicine at O'Neal Comprehensive Cancer Center, University of Alabama at Birmingham

Session #: Title: 634, Myeloproliferative Syndromes: Clinical and Epidemiological: Poster II

Date and Time: Sunday, December 13, 2026, 6:00 - 8:00 PM CDT

Location: MCCNO – Hall F

Poster #: 3771

About Sapablursen (ONO-0530)

Sapablursen, an antisense oligonucleotide, is designed to reduce the production of TMPRSS6, a negative regulator of hepcidin. Reducing TMPRSS6 is intended to increase hepcidin, the key regulator of iron homeostasis. By increasing the production of hepcidin, sapablursen has the potential to positively impact polycythemia vera by decreasing hematocrit, reducing the need for phlebotomy, and improving quality of life.

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, heart attack, stroke, and death. The primary treatment goal in PV is to maintain blood hematocrit levels <45% to prevent thrombotic events and alleviate burdensome symptoms, such as severe fatigue, difficulty concentrating, night sweats, and pruritus. Despite available treatment options, some patients continue to experience burdensome symptoms or inadequate hematocrit control.

About Deciphera Pharmaceuticals Inc.

Deciphera, a member of Ono Pharmaceutical Co., Ltd., is a biopharmaceutical company focused on discovering, developing, and commercializing innovative medicines to help people live longer, healthier lives. We harness cutting-edge technologies including advanced kinase-inhibitor science, data analytics, and AI-enabled drug discovery to accelerate breakthroughs in our key focus areas; oncology, immunology & inflammation and neurology. Headquartered in Waltham, MA with research facilities in Lawrence, KS, Deciphera is leading Ono's expansion in the U.S. and Europe with both commercial products and a robust pipeline of clinical programs with first- or best-in-class potential. For more information, visit www.deciphera.com and follow us on LinkedIn and X (@Deciphera).

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