



Japan MHLW approves Bylvay® (odevixibat) for patients living with pruritus due to Progressive Familial Intrahepatic Cholestasis (PFIC)

TOKYO, JAPAN, 19 September 2025 – Jadeite Medicines Inc., (Jadeite Medicines) a clinical stage biopharmaceutical company headquartered in Tokyo, Japan (President & CEO, Eiichi Takahashi) today confirmed that the Ministry of Health, Labour and Welfare (MHLW) of Japan has granted an approval to IPSEN Co., Ltd., headquartered in Tokyo, Japan (President and Representative Director: Robyn Busby, a wholly-owned subsidiary of Ipsen (Euronext: IPN; ADR: IPSEY)) for Bylvay® (odevixibat) for the treatment of pruritus in patients with Progressive Familial Intrahepatic Cholestasis (PFIC). Bylvay is a once-daily, non-systemic ileal bile acid transport inhibitor (IBATi) that acts locally in the small intestine and has minimal systemic exposure.

Based on a strategic collaboration between Jadeite Medicines and Albireo Pharma Inc., a wholly-owned subsidiary of Ipsen, Jadeite Medicines is responsible for clinical development of Bylvay in Japan. Jadeite Medicines completed the phase 3 trial (A4250-J005) and prepared the dossier required for the New Drug Application (NDA) for the indication of PFIC in Japan.

The NDA for this indication was filed by IPSEN Co., Ltd., based on the strategic collaboration between Jadeite Medicines and Albireo Pharma Inc. IPSEN Co., Ltd. will also be responsible for commercialization of the medicine.

With Bylvay now approved, Jadeite Medicines is committed to contributing to the treatment of patients suffering from PFIC.

In addition, Jadeite Medicines is currently conducting a phase 3 clinical trial (A4250-J012) in Japan to evaluate the efficacy and safety of odevixibat for the treatment of Alagille Syndrome (ALGS) patients.

▽Progressive Familial Intrahepatic Cholestasis (PFIC)

PFIC is a spectrum of autosomal recessive genetic disorders in which cholestasis may lead to end-stage liver disease. PFIC is designated as an intractable disease (designation number 338) by the MHLW in Japan. PFIC is estimated to affect between one in every 50,000 to 100,000 children born worldwide. PFIC represents 10% to 15% of cases of cholestasis in children. In Japan, estimated 100 patients suffer from PFIC.

Symptoms develop early with median age at onset of symptoms of approximately 3 months and jaundice, pale stools, and intractable pruritus become apparent at 3-4 months of age, accompanied by hepatosplenomegaly and growth failure.

▽Bylvay

Bylvay is a once-daily, potent non-systemic ileal bile acid transport inhibitor (IBATi) that acts locally within the small intestine.

Bylvay is approved and marketed by Ipsen in the United States, Europe and other territories for the treatment of cholestatic pruritus in patients with ALGS and patients with PFIC. Bylvay has also received orphan drug designation for the treatment of biliary atresia and primary biliary cirrhosis in the U.S. and Europe, and the Phase 3 study (BOLD trial) in patients with biliary atresia is ongoing overseas.

View Japan product information here: <https://bylvayhcp.jp/home/>

▽Jadeite Medicines Inc.

Jadeite Medicines Inc. is a biopharmaceutical company established in 2020 to fulfill the unmet medical needs of patients in Japan by introducing and developing innovative medicines from around the world. Jadeite Medicines team consists of highly experienced professionals in the field of clinical development, regulatory, CMC and business development. Jadeite Medicines continues to search for first-in-class and best-in-class medicines from around the world to create a truly innovative product portfolio. Jadeite Medicines is backed by CBC Group, Asia's largest healthcare-dedicated investment firm. For more information, visit

<https://www.jadeitemedicines.co.jp/en/index.html>