



Jadeite Medicines Receives Orphan Drug Designation for Odevixibat in Progressive Familial Intrahepatic Cholestasis (PFIC) from the Ministry of Health, Labour and Welfare of Japan and will also initiate Phase 3 Trial of Odevixibat in PFIC in Japan

TOKYO, JAPAN, 21st June 2023 - Jadeite Medicines Inc., (Jadeite Medicines) a clinical stage biopharmaceutical company headquartered in Tokyo, Japan (President & CEO, Eiichi Takahashi) has received Orphan Drug Designation for odevixibat for the expected indication of PFIC from the Ministry of Health, Labour and Welfare (MHLW) of Japan. Jadeite Medicines has also announced initiation of a phase 3 trial in PFIC for patients in Japan. PFIC is a rare and devastating disorder affecting young children with no approved treatment in Japan.

This Phase 3 trial will evaluate the efficacy and safety of an oral dosage of odevixibat in Japanese PFIC patients. Odevixibat is marketed by Ipsen Pharma (Ipsen) in the United States for the treatment of pruritus in all subtypes of PFIC and in Europe for the treatment of all subtypes of PFIC.

Through the development of odevixibat, Jadeite Medicines aspires to contribute to the treatment of patients suffering from PFIC. Jadeite Medicines commits to the health and quality of life for patients through our relentless R&D in drug development in order to fulfill patient unmet needs.

▽Progressive Familial Intrahepatic Cholestasis (PFIC)

PFIC is a spectrum¹⁻⁴ of autosomal recessive genetic disorders in which cholestasis may lead to end-stage liver disease.⁵ The estimated global incidence of PFIC is 1 in 100,000 live births.⁵ Currently in the U.S., it is estimated that there are 500 PFIC patients who may be eligible for IBATi treatment. Subtypes PFIC1, PFIC2 and PFIC3 are the most common.⁵ In addition, other rare forms of PFIC exist with varying degrees of cholestasis.⁶ Patients with PFIC have impaired bile flow, or cholestasis, and the resulting bile build-up in liver cells causes liver disease and symptoms. The most debilitating symptom of PFIC is pruritus (itching), which may be so severe that it leads to skin mutilation, loss of sleep, irritability, poor attention and impaired school performance.⁴ Up to 80% of PFIC patients suffer from severe pruritus, associated with abrasions, skin mutilation, hemorrhage or scarring.⁷ PFIC is designated as an intractable disease (designation number 338) by the MHLW in Japan. In Japan, an estimated 100 patients suffer from PFIC. Currently there are no treatments except for symptomatic treatments in Japan and without conservative treatment patients will require surgical options including liver transplantation.

▽Odevixibat

Odevixibat is being developed by Albireo Pharma, Inc., which was acquired by Ipsen Pharmaceutical in March 2023. Albireo Pharma and Jadeite Medicines entered into an exclusive licensing agreement for the development and commercialization of odevixibat in Japan, in October 2021.

Odevixibat is a once-daily, potent non-systemic ileal bile acid transport inhibitor (IBATi) that acts locally within the small intestine. Odevixibat is the first ever approved treatment for all subtypes of PFIC for patients above the age of three months in the U.S. (for the treatment of pruritus). Odevixibat is also approved by the EMA and the Medicines and Healthcare Products Regulatory Agency of the United Kingdom for all subtypes of PFIC for patients above the age of six months and is being widely commercialized in Europe. Odevixibat has been granted orphan drug designation in the U.S. and Europe. Ipsen is also filing for regulatory approval for Alagille syndrome in the U.S. based on their successful ASSERT Phase 3 clinical trial. Ipsen is also studying the use of odevixibat in other rare pediatric cholestatic liver diseases with the BOLD Phase 3 clinical trial in patients with biliary atresia where topline data from the BOLD trial is expected in 2024.

▽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 www.jadeitemedicines.co.jp.

▽Ipsen

Ipsen is a global, mid-sized biopharmaceutical company focused on transformative medicines in Oncology, Rare Disease and Neuroscience. With total sales of €3.0bn in FY 2022, Ipsen sells medicines in over 100 countries. Alongside its external-innovation strategy, the Company's research and development efforts are focused on its innovative and differentiated technological platforms located in the heart of leading biotechnological and life-science hubs: Paris-Saclay, France; Oxford, U.K.; Cambridge, U.S.; Shanghai, China. Ipsen has around 5,000 colleagues worldwide and is listed in Paris (Euronext: IPN) and in the U.S. through a Sponsored Level I American Depositary Receipt program (ADR: IPSEY). For more information, visit ipsen.com.

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