Jadeite Medicines Receives Orphan Drug Designation with Odevixibat for Treatment of Alagille Syndrome (ALGS) from Ministry of Health, Labour and Welfare of Japan

TOKYO, JAPAN, 25th December 2024 - 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 ALGS from the Ministry of Health, Labour and Welfare (MHLW) of Japan.

Odevixibat is already approved for cholestatic pruritus in patients with ALGS and patients with progressive familial intrahepatic cholestasis (PFIC) in the United States and European countries and is marketed by Ipsen.

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

▽Alagille Syndrome (ALGS)

ALGS is an inherited rare, genetic disorder that can affect multiple organ systems in the body including the liver, heart, skeleton, eyes and kidneys. Liver damage may result from having fewer than normal, narrowed or malformed bile ducts, which leads to toxic bile acid build-up, which in turn can cause scarring and progressive liver disease. Approximately 95% of patients with the condition present with chronic cholestasis, usually within the first few months of life and as many as 88% also present with severe, intractable pruritus. The estimated global incidence of ALGS is 3 in 100,000 live births.

ALGS is designated as an intractable disease (designation number 297) by the MHLW in Japan. Based on the results of a nationwide survey, it is estimated that the number of patients in Japan is about 200-300.

▽Odevixibat

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

Odevixibat 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.

Odevixibat 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 III BOLD trial in patients with biliary atresia is ongoing overseas.

▽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 biopharmaceutical company with a focus on bringing transformative medicines to patients in three therapeutic areas: Oncology, Rare Disease and Neuroscience.

Ipsen's pipeline is fueled by external innovation and supported by nearly 100 years of development experience and global hubs in the U.S., France and the U.K. Ipsen's teams in more than 40 countries and partnerships around the world enable Ipsen to bring medicines to patients

in more than 80 countries.

Ipsen 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.