Press release

Intended for international media and investor audiences only

Ipsen presents long-term elafibranor efficacy and itch-related quality of life data in patients with primary biliary cholangitis

- ***New data from the ELATIVE® Phase III trial show 70% of patients treated with elafibranor achieved composite endpoint of slowing disease progression measured by biochemical response after 78-weeks***
- ***Data from the itch domain of the PBC-40 and 5-D Itch questionnaires shows the potential of elafibranor to improve itch-related quality of life in patients with moderate-to-severe pruritus***
- ***Significant unmet need remains for new treatment options in primary biliary cholangitis that control disease progression and debilitating symptoms impacting quality of life***

PARIS, FRANCE, DD June 2024 Ipsen (Euronext: IPN; ADR: IPSEY) today announced new late-breaking data at the European Association for the Study of the Liver (EASL) Congress demonstrating the enduring efficacy of elafibranor in managing disease progression after 78 weeks of treatment. In the variable double-blind period of the ELATIVE® Phase III trial in primary biliary cholangitis (PBC), the potential for elafibranor to improve itch-related quality of life (QoL) as measured by the itch domain of the PBC-40 and the 5-D Itch questionnaire was also demonstrated. Elafibranor is a novel, potential first-in-class, PPAR agonist. It is currently under review by the U.S. Food and Drug Administration, the European Medicines Agency and the UK Medicines and Healthcare Products Regulatory Authority.

"There are a significant proportion of people living with PBC who experience worsening disease and debilitating symptoms despite being on treatment. These long-term data from the Phase III ELATIVE study further demonstrate the potential for elafibranor to provide an effective treatment option for these patients," said Sandra Silvestri, M.D. Executive Vice President and Chief Medical Officer, Ipsen. "A lack of effective management can lead to advanced forms of the disease where liver transplantation may be the only option. Transplants are not trivial, so we must and can do better to preserve native liver function for people living with PBC."

Data presented at EASL for patients who had their week-78 double-blind visit (30 patients receiving elafibranor and 13 patients receiving placebo) demonstrated the efficacy of elafibranor was sustained after 78 weeks of treatment with 70% of patients on elafibranor meeting the composite endpoint of biochemical response versus 0% on placebo. Biochemical response was defined as alkaline phosphatase (ALP) <1.67 x upper limit of normal (ULN), an ALP decrease ≥ 15 percent and total bilirubin (TB) ≤ ULN. ALP and bilirubin are important predictors of PBC disease progression. Reductions in levels of both can indicate reduced liver injury and improved liver function. ALP normalization for patients on elafibranor was sustained out to week-78 as well as across other important biomarkers of liver injury, including total bilirubin and gamma glutamyl transferase.[1]

New patient-reported outcome data from ELATIVE at week 52 were also presented, demonstrating the potential beneficial effect of elafibranor on itch-related quality of life, including sleep and functioning. Treatment with elafibranor led to greater reductions in 5-D Itch score which comprises five domains (degree, duration, dimension, disability and distribution) versus placebo. A clinically meaningful reduction in the itch domain of PBC-40 for elafibranor versus placebo was also observed, with a greater proportion of patients treated with elafibranor experiencing improvement in itch-related quality of life. These include across measures of severity of itching, sleep disturbance and emotional impact of itching, versus placebo. In the 5-D Itch domain of duration, reduced itching was reported by 58% of patients receiving elafibranor at week 52, compared with 27% on placebo. Additionally, 80% of patients receiving elafibranor improved to no sleep disturbance or only occasional delay, compared with 30% on placebo. The improvements in 5-D Itch and PBC-40 Itch emphasize the potential of elafibranor to reduce both the severity of PBC symptoms and their impact on QoL.[2]

"When you have a patient with PBC, it's vital to manage disease progression, to prevent or delay liver damage or failure. You also want to provide relief from distressing symptoms because they can have a very detrimental impact on quality of life," said Dr. Christopher Bowlus, Professor of Gastroenterology and Hepatology, University of California Davis, U.S. "These new data from ELATIVE provide further evidence that elafibranor has the potential to address the two priority treatment goals by demonstrating longer-term improvements in the prognostic markers of disease progression, as well as potential improvements in pruritus-symptom severity and impacts on the quality of life."

PBC is a rare, progressive, autoimmune cholestatic liver disease, in which the body attacks and gradually destroys the liver's small bile ducts.[3] If left untreated, bile and toxins can build up, leading to scarring of the liver and eventual liver failure.[3-5] Symptoms of PBC can have a substantial impact on a person's QoL, including fatigue and itching.[6,7] However, while some people living with PBC may not display symptoms, they remain at risk of disease progression and liver damage, making active disease management vital.[8]

Ipsen also presented new data from its growing rare cholestatic liver disease portfolio at EASL, including data on its treatment for progressive familial intrahepatic cholestasis and Alagille syndrome.

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About ELATIVE

ELATIVE is a multi-center, randomized, double-blind, placebo-controlled Phase III clinical trial, with an open-label long-term extension (NCT04526665). ELATIVE is evaluating the efficacy and safety of elafibranor 80mg once daily versus placebo for the treatment of patients with PBC with an inadequate response or intolerance to ursodeoxycholic acid (UDCA), the existing first-line therapy for PBC. The trial enrolled 161 patients who were randomized 2:1 to receive elafibranor 80mg once daily or placebo. Patients with an inadequate response to UDCA would continue to receive UDCA in combination with elafibranor or placebo, while patients unable to tolerate UDCA would receive only elafibranor or placebo. Patients continued their assigned treatment after Week 52 until all patients had completed their treatment or for a maximum of 104 weeks. Data was also collected during this period, and additional analyses were conducted with a focus on Week 78.

About Ipsen

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

Our 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. Our teams in more than 40 countries and our partnerships around the world enable us 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.

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