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PRESS RELEASE

U.S. FDA approves Bylvay® for patients living with cholestatic pruritus due to Alagille syndrome

- Approval heralds the second rare cholestatic liver disease indication for Bylvay in the U.S. after progressive familial intrahepatic cholestasis related pruritus in 2021
- Immediate U.S. commercial launch and availability for eligible patients
- ASSERT clinical study demonstrated efficacy of Bylvay in improvement of pruritus. Also showed improvement in certain sleep disturbances and reduction in bile acids – which were secondary endpoints – with a low drug-related diarrhea rate in patients with Alagille syndrome
- Committee for Medicinal Products for Human Use opinion expected in Q2 2023 with final European Medicines Agency decision in second half of 2023

PARIS, FRANCE, 26 May 2023 – Ipsen (Euronext: IPN: ADR: IPSEY) today announced that the U.S. Food and Drug Administration (FDA) has approved Bylvay® (odevixibat) for the treatment of cholestatic pruritus in patients from 12 months of age with Alagille syndrome (ALGS). 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. Bylvay was approved as the first drug treatment option for patients living with cholestatic pruritus due to progressive familial intrahepatic cholestasis (PFIC) in the U.S., and for the treatment of PFIC in Europe, in 2021. Bylvay is immediately available via prescription for eligible ALGS patients.

“Today’s approval of Bylvay in a second indication allows patients and physicians to access an additional treatment option that has the potential to improve the management of pruritus or intense itch, in this distressing condition that tends to affect young children,” said Howard Mayer, Executive Vice President and Head of Research and Development for Ipsen. “We are proud to have achieved FDA approval for Bylvay as a treatment for ALGS in the U.S. and we are committed to making it available to many more eligible patients across the world.”

Positive data from the Phase III ASSERT study, presented at the 2022 American Association for Study of Liver Diseases congress, demonstrated that Bylvay provided highly statistically significant and clinically meaningful sustained improvements in pruritus, starting early after initiation of treatment. More than 90% of patients were pruritus responders (≥ 1 point change at any time during 24 weeks). The overall incidence of treatment-emergent adverse events was similar to placebo. No patients discontinued the study and 96% of patients rolled over into the open-label extension study.

“Physicians urgently need more options to treat patients with Alagille syndrome and this approval from the U.S. FDA spotlights the robustness of the Phase III ASSERT clinical study results,” said Nadia Ovchinsky, MD, Chief of the Division of Gastroenterology and Hepatology, Hassenfeld Children's Hospital at NYU Langone and ASSERT Principal Investigator. “The ASSERT study showed that Bylvay reduced pruritus associated with ALGS, which is so common among this patient population and one of the leading indications for a liver transplant.”

Roberta Smith, President, Alagille Syndrome Alliance said: “As an advocate for families impacted by Alagille syndrome, it is such a blessing to know physicians now have another drug treatment option for the debilitating pruritus that affects so many Alagille patients. I know personally the terrible impact of this rare disease on a child; this approval will help to alleviate the pruritus burden for more patients.”

Ipsen has also submitted Bylvay to the European Medicines Agency (EMA), seeking authorization for ALGS, with Committee for Medicinal Products for Human Use opinion expected in Q2 2023 and final EMA regulatory decision anticipated in second half of 2023. Bylvay has received orphan exclusivity for the treatment of PFIC, and Orphan Drug Designations for the treatment of ALGS and biliary atresia, in the

U.S. and Europe. Bylvay is already approved in the U.S. for the treatment of pruritus in patients aged three months and older with all types of PFIC, and in Europe for the treatment of all types of PFIC in patients aged six months or older. In a third indication, the rare pediatric cholestatic liver disease, biliary atresia, Bylvay is in late-stage development with the Phase III BOLD trial.

ENDS

About Bylvay® (odevixibat)
Bylvay is a potent, once-daily, non-systemic IBATi that acts locally in the small intestine and has minimal systemic exposure. It is approved in the U.S. for the treatment of pruritus in patients three months of age and older with PFIC, where it has orphan exclusivity. Bylvay was first launched as a treatment option for patients with PFIC in the U.S. in 2021, where it is supported by a program designed to assist with access to treatment and patient support. Bylvay is also approved in the E.U. for the treatment of PFIC in patients aged six months or older. It has launched in over nine countries and has secured public reimbursement across several major markets including Germany, Italy, the U.K., France and Belgium.

View full U.S. prescribing information here: label (fda.gov)
View full E.U. prescribing information here: Bylvay, INN-odevixibat (europa.eu)

Important Safety Information

- PFIC: The most common adverse reactions are diarrhea, liver test abnormalities, vomiting, abdominal pain, and fat-soluble vitamin deficiency.
- ALGS: The most common adverse reactions are diarrhea, abdominal pain, hematoma, and weight decrease.
- Liver Test Abnormalities: Patients should obtain baseline liver tests and monitor during treatment. Dose reduction or treatment interruption may be required if abnormalities occur. For persistent or recurrent liver test abnormalities, consider treatment discontinuation.
- Diarrhea: Treat dehydration. Treatment interruption or discontinuation may be required for persistent diarrhea.
- Fat-Soluble Vitamin (FSV) Deficiency: Patient should obtain baseline vitamin levels and monitor during treatment. Supplement if deficiency is observed. If FSV deficiency persists or worsens despite FSV supplementation, discontinue treatment.

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.[1] 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.[2,3] The estimated global incidence of ALGS is 3 in 100,000 live births.[4] Currently in the U.S., it is estimated that there are 1,300 patients who may be eligible for IBATi treatment.

ASSERT Phase III Clinical Trial Data
ASSERT is a double-blind, randomized, placebo-controlled trial designed to evaluate the safety and efficacy of 120 µg /kg/day Bylvay for 24 weeks in relieving pruritus in patients with ALGS with 32 sites across North America, Europe, Middle East, and Asia Pacific. The trial enrolled patients aged 0 to 17 years of age with a genetically confirmed diagnosis of ALGS. In the primary analysis, the study met the primary endpoint showing highly statistically significant improvement in pruritus as measured by the PRUCISION Observer-Reported Outcome scratching score (0-4 point scale), from baseline at month 6 (weeks 21 to 24), compared to the placebo arm (p=0.002). More than 90% of patients were pruritus responders (≥ 1 point change at any time during 24 weeks). The study also met the key secondary endpoint showing a highly statistically significant reduction in serum bile acid concentration from baseline to the average of weeks 20 and 24 (compared to the placebo arm p=0.001). Statistically significant improvements in multiple sleep parameters were observed as early as weeks 1-4 compared to patients on placebo with continued improvement through week 24. In the study, there were no patient

discontinuations and 96% of patients rolled over into the open-label extension study. Bylvay had an overall adverse event incidence similar to placebo and a low incidence of drug-related diarrhea (11.4% vs. 5.9% placebo).

About 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 5,400 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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Ipsen's forward-looking statements
The forward-looking statements, objectives and targets contained herein are based on Ipsen's management strategy, current views and assumptions. Such statements involve known and unknown risks and uncertainties that may cause actual results, performance or events to differ materially from those anticipated herein. All of the above risks could affect Ipsen's future ability to achieve its financial targets, which were set assuming reasonable macroeconomic conditions based on the information available today. Use of the words 'believes', 'anticipates' and 'expects' and similar expressions are intended to identify forward-looking statements, including Ipsen's expectations regarding future events, including regulatory filings and determinations. Moreover, the targets described in this document were prepared without taking into account external-growth assumptions and potential future acquisitions, which may alter these parameters. These objectives are based on data and assumptions regarded as reasonable by Ipsen. These targets depend on conditions or facts likely to happen in the future, and not exclusively on historical data. Actual results may depart significantly from these targets given the occurrence of certain risks and uncertainties, notably the fact that a promising medicine in early development phase or clinical trial may end up never being launched on the market or reaching its commercial targets, notably for

regulatory or competition reasons. Ipsen must face or might face competition from generic medicine that might translate into a loss of market share. Furthermore, the research and development process involves several stages each of which involves the substantial risk that Ipsen may fail to achieve its objectives and be forced to abandon its efforts with regards to a medicine in which it has invested significant sums. Therefore, Ipsen cannot be certain that favorable results obtained during preclinical trials will be confirmed subsequently during clinical trials, or that the results of clinical trials will be sufficient to demonstrate the safe and effective nature of the medicine concerned. There can be no guarantees a medicine will receive the necessary regulatory approvals or that the medicine will prove to be commercially successful. If underlying assumptions prove inaccurate or risks or uncertainties materialize, actual results may differ materially from those set forth in the forward-looking statements. Other risks and uncertainties include but are not limited to, general industry conditions and competition; general economic factors, including interest rate and currency exchange rate fluctuations; the impact of pharmaceutical industry regulation and healthcare legislation; global trends toward healthcare cost containment; technological advances, new medicine and patents attained by competitors; challenges inherent in new-medicine development, including obtaining regulatory approval; Ipsen's ability to accurately predict future market conditions; manufacturing difficulties or delays; financial instability of international economies and sovereign risk; dependence on the effectiveness of Ipsen's patents and other protections for innovative medicines; and the exposure to litigation, including patent litigation, and/or regulatory actions. Ipsen also depends on third parties to develop and market some of its medicines which could potentially generate substantial royalties; these partners could behave in such ways which could cause damage to Ipsen's activities and financial results. Ipsen cannot be certain that its partners will fulfil their obligations. It might be unable to obtain any benefit from those agreements. A default by any of Ipsen's partners could generate lower revenues than expected. Such situations could have a negative impact on Ipsen's business, financial position or performance. Ipsen expressly disclaims any obligation or undertaking to update or revise any forward-looking statements, targets or estimates contained in this press release to reflect any change in events, conditions, assumptions or circumstances on which any such statements are based, unless so required by applicable law. Ipsen's business is subject to the risk factors outlined in its registration documents filed with the French Autorité des Marchés Financiers. The risks and uncertainties set out are not exhaustive and the reader is advised to refer to Ipsen's latest Universal Registration Document, available on ipsen.com.

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