Press Release

Ipsen provides update on Phase III BOLD trial in biliary atresia

PARIS, FRANCE, 24 JULY 2026 – Ipsen (Euronext: IPN; ADR: IPSEY) announced today that the Phase III BOLD trial evaluating Bylvay versus placebo for patients living with biliary atresia (BA) who have already undergone a Kasai hepatoportoenterostomy (HPE) did not meet the primary endpoint of improvement in native liver survival vs placebo. Current topline data is in line with the well-established safety profile of odevixibat in approved indications. Characterized as a rapidly progressive and complex condition, BA is the leading cause of paediatric liver transplant, with no approved therapeutic options beyond Kasai HPE or liver transplant surgery.

"Biliary atresia is a rare and serious liver disease that affects babies. Progressing rapidly and with no effective medical treatments, many children develop severe liver damage and biliary atresia remains the number 1 cause of liver transplantation in children, often before the age of 2. Research remains limited, leaving patients, families and clinicians with very few therapeutic options," said lead investigator Dr Saul J. Karpen MD PhD FAASLD, Pediatric Hepatologist and Chief Scientific Officer, Stravitz-Sanyal Institute for Liver Disease and Metabolic Health, Virginia Commonwealth University. "As the first global Phase III trial in biliary atresia, BOLD has generated the most comprehensive dataset ever assembled in this disease. Although the study did not meet its primary endpoint, the commitment of participating children and families has produced valuable insights that will deepen our understanding of biliary atresia and provide a crucial basis for future research and patient care. As biliary atresia is a heterogeneous disease with variable clinical presentation and progression, further analyses of this comprehensive dataset may provide important insights into disease biology and help us better understand whether outcomes differ across patient subgroups."

"This outcome is disappointing for patients living with this serious disease and their families," said Christelle Huguet, PhD, EVP Head of R&D, Ipsen. "These results reflect the significant challenge biliary atresia presents as a complex, rare paediatric cholestatic liver disease which has so far evaded all therapeutic attempts beyond surgery. We would like to sincerely thank the patients, caregivers, investigators, and the broader biliary atresia community who dedicated their time and effort to the BOLD trial. Their commitment has been instrumental in helping advance our understanding of biliary atresia and the needs of those living with this challenging disease."

About Bylvay (odevixibat)

Odevixibat is a once-daily selective and potent ileal bile acid transport (IBAT) inhibitor that reduces bile acid reabsorption in the intestine. Through IBAT inhibition, the bile acids are instead diverted and excreted with faeces. Bylvay® (odevixibat) is approved in the U.S. as the first treatment for cholestatic pruritus in Progressive Familial Intrahepatic Cholestasis (PFIC) across all types, and for pruritus in patients with Alagille Syndrome (ALGS). In the EU Bylvay is approved for the treatment of PFIC, with orphan exclusivity granted, and is additionally marketed as KAYFANDA® for ALGS.

About the BOLD Phase III trial

BOLD is a Phase III randomized, double-blind, placebo-controlled, trial (NCT04336722) investigating the efficacy and safety of odevixibat compared to placebo in patients with biliary atresia who have undergone a Kasai hepatoportoenterostomy (HPE). It is the largest trial evaluating disease modification in biliary atresia. The trial enrolled 254 patients across 19 countries who had undergone Kasai HPE surgery within the first 90 days of life. Patients received oral odevixibat 120mcg/kg/day or placebo once daily for up to 104 weeks. The primary endpoint was native liver survival, defined as

time from randomization to first occurrence of liver transplant or death at week 104. An open-label extension study currently ongoing (BOLD-EXT) is evaluating the longer-term safety and efficacy of odevixibat in patients who have completed the BOLD trial. A decision regarding the continuation of patients in the open-label extension will be made following a comprehensive review of the full trial data.

About biliary atresia

Biliary atresia (BA) is a rare, serious pediatric cholestatic liver disease affecting 1 in 5,000-20,000 newborns in which the bile ducts inside and/or outside the liver are blocked, absent, or scarred, preventing bile from draining from the liver into the intestine. Bile then builds up in the liver, causing progressive inflammation, fibrosis, cirrhosis and ultimately liver failure if untreated. The condition appears in newborns and young infants, typically within the first weeks to months of life, often through persistent jaundice, pale stools and dark urine. Biliary atresia is the most common cause of pediatric liver transplantation worldwide. While an early Kasai HPE surgical procedure can restore bile flow and delay disease progression, it does not always cure the condition and many patients will require a liver transplant. Currently there are no approved medical treatments options for BA.

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 internal and 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 100 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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