Media update

Ipsen to present new data across four rare liver diseases at EASL, including late-breaking data in PBC and PSC

- ***Seven abstracts, including three late-breaking presentations, will be shared on new IQIRVO® (elafibranor) and Bylvay®/Kayfanda® (odevixibat) data, reinforcing the strong clinical value across Ipsen's rare liver disease portfolio where unmet need is high and treatments are few***

- ***In two late-breaking presentations, IQIRVO's effect on fatigue and its relationship with pruritus in patients with primary biliary cholangitis (PBC) is shared, alongside proteomic results demonstrating IQIRVO impacts on inflammation, biochemistry, fibrosis and symptom-associated markers***

- ***IQIRVO's efficacy benefits are supported by long-term stabilization of non-invasive markers of liver fibrosis data and a new bone health analysis reinforces its safety profile in PBC***

- ***Simultaneous publication in Journal of Hepatology and oral late-breaking presentation of Phase II ELMWOOD results evaluates safety and efficacy of elafibranor in primary sclerosing cholangitis***

- ***Longer-term Bylvay/Kayfanda efficacy and safety data presented for progressive familial intrahepatic cholangitis and Alagille syndrome, two rare liver diseases***

PARIS, FRANCE, 6 May 2025 – Today, Ipsen (Euronext: IPN; ADR: IPSEY) announced that seven abstracts with new data from its rare liver disease portfolio will be presented at the European Association for the Study of the Liver (EASL) congress, 7 – 10 May, in Amsterdam, Netherlands. These include two late-breaking abstracts selected for poster presentation from Ipsen's IQIRVO® (elafibranor) in primary biliary cholangitis (PBC) and one late-breaking abstract selected for oral presentation and simultaneous publication in the *Journal of Hepatology: Safety and efficacy of elafibranor in primary sclerosing cholangitis: The ELMWOOD phase II randomized controlled trial*, on elafibranor in primary sclerosing cholangitis (PSC). Two other abstracts describing the effect of IQIRVO non-invasive markers of fibrosis and bone health in PBC were also selected for poster presentation.

In addition, two abstracts with data on odevixibat known in the EU as Bylvay® for PFIC and Kayfanda® for ALGS, will be shared as poster presentations. These data are from the open label extension studies in progressive familial intrahepatic cholangitis (PFIC) and Alagille syndrome (ALGS) respectively.

Upcoming presentations at EASL will reinforce the long-term efficacy benefits and safety profile of IQIRVO with data from the Phase III ELATIVE study and the ongoing open-label extension in patients with PBC:

- Data from the ongoing ELATIVE open-label extension demonstrate stabilization of non-invasive markers of fibrosis after two-years of treatment, suggesting IQIRVO may slow worsening of fibrosis in patients with PBC.

- An analysis of the effect of IQIRVO on bone mineral density and biomarkers of bone turnover after 52 weeks in the ELATIVE study suggests there is no negative impact on bone health in patients with PBC taking IQIRVO.

New long-term data from the open-label extension studies with Bylvay in PFIC and Kayfanda in ALGS provide a growing body of evidence for these rare liver diseases:

- Data from PEDFIC-2 on patients 18 years and older with PFIC treated with Bylvay demonstrated clinically meaningful improvements in pruritus and reductions in serum bile acid. This further characterizes the clinical benefits of long-term Bylvay therapy for a disease associated with paediatrics and commonly misdiagnosed in adults.

- For patients with ALGS, efficacy and safety outcomes were studied for patients aged 10 years and older from the ASSERT-EXT long-term extension study. Bylvay, known as Kayfanda in EU for ALGS, was found to be well-tolerated, with patients achieving clinically meaningful improvements in pruritus, reductions in serum bile acid and improvements in sleep outcomes out to 72 weeks.

With two molecules approved for three different rare cholestatic liver diseases and a pipeline with two additional late-stage indications, Ipsen is leading research and development for treatments where unmet need is high and treatments are often few or do not exist. By focusing on conditions like PSC, PBC, PFIC and ALGS, Ipsen aims to improve patient outcomes and quality of life. The company's commitment is reflected in its rigorous clinical trials and ongoing efforts to develop effective treatments. This demonstrate Ipsen's unwavering support for patients affected by these challenging and often debilitating diseases.

Data presentations at EASL

Poster or Oral#	Full title	Authors
Late-breaking oral abstract LB25222 [OS-089] Saturday 10 May 2025 11:15–11:30	Elafibranor for primary sclerosing cholangitis: The ELMWOOD phase II randomised controlled trial	Cynthia Levy, et al
Late-breaking poster abstract LB25202 [LBP-025] Wednesday 7 May 2025 13:15–14:45	Elafibranor impacts inflammatory, fibrotic and symptom-associated markers in patients with primary biliary cholangitis: Proteomic results from the ELATIVE® trial	Mark G. Swain, et al
Late-breaking poster abstract LB25220 {LBP-027] Wednesday 7 May 2025 13:15–14:45	Elafibranor improves fatigue versus placebo in patients with primary biliary cholangitis, with limited correlation with pruritus: Analysis from the phase III ELATIVE® trial	David E. Jones, et al
Poster, abstract 909 [THU-313] Thursday 8 May 2025 12:30–14:00	Treatment with elafibranor has no impact on bone health in patients with primary biliary cholangitis	Jörn M. Schattenberg, et al
Poster, abstract 89 [THU-333] Thursday 8 May 2025 12:30–14:00	Non-invasive tests for liver fibrosis are stable in patients with primary biliary cholangitis with two years of treatment with elafibranor	Marlyn J. Mayo, et al
Poster, abstract 810 [THU-306] Thursday 8 May 2025 12:30–14:00	Efficacy and safety of odevixibat in adult patients with progressive familial intrahepatic cholestasis: Results from the 72-week PEDFIC2 phase III, open-label study	Henkjan J. Verkade, et al

Poster, abstract 1029 [THU-342] Thursday 8 May 2025 12:30–14:00	Efficacy and safety of odevixibat in patients ≥10 years with Alagille syndrome: Results from the 72-week ASSERT-EXT phase III, open-label extension study	Nadia Ovchinsky, et al

ENDS

About IQIRVO® (elafibranor)

Iqirvo (pronounced EYE-KER-VO) is an oral, once-daily, peroxisome proliferator-activated receptor (PPAR) agonist, which exerts an effect on PPARα and PPARδ. Activation of PPARα and PPARδ decreases bile toxicity and improves cholestasis by modulating bile acid synthesis, detoxification and transporters. Activation of PPARα and PPARδ also has anti-inflammatory effects by acting on different pathways. In 2019, Iqirvo was granted Breakthrough Therapy Designation by the U.S Food and Drug Administration (FDA) in adults with PBC who have an inadequate response to ursodeoxycholic acid (UDCA), the existing first-line therapy for PBC. Iqirvo was granted U.S. FDA accelerated approval in June 2024, conditional approval by the EMA in September 2024 and UK Medicines and Healthcare products Regulatory Agency (MHRA) in October 2024, for the treatment of primary biliary cholangitis (PBC) in combination with ursodeoxycholic acid (UDCA) in adults who have an inadequate response to UDCA, or as monotherapy in patients unable to tolerate UDCA. The FDA, EMA and MHRA approvals are contingent on the further verification of clinical benefit. Iqirvo is currently in regulatory processes with other authorities. Iqirvo (elafibranor) was developed by GENFIT. Ipsen licensed the exclusive worldwide rights (except China, Hong Kong, Taiwan and Macau) to elafibranor from GENFIT in 2021.

About Bylvay® /Kayfanda® (odevixibat)

Bylvay (known as Kayfanda® in E.U. in ALGS) (odevixibat) is a once-daily non-systemic ileal bile acid transport (IBAT) inhibitor. Odevixibat was approved in July 2021 under exceptional circumstances in the E.U. under the brand name Bylvay®, as the first drug treatment option for all types of progressive familial intrahepatic cholestasis (PFIC) in patients aged 6 months or older, and in June 2021 in the U.S. under the brand name Bylvay®, as the first drug treatment option for patients 3 months of age and older living with cholestatic pruritus due to PFIC. Bylvay has received orphan exclusivity for the treatment of PFIC in the E.U. and in the U.S.

Odevixibat was approved in September 2024 under exceptional circumstances in the E.U. under the brand name Kayfanda® for the treatment of cholestatic pruritus in Alagille syndrome (ALGS) in patients aged 6 months or older. In June 2023 Bylvay was approved in the U.S. for the treatment of cholestatic pruritus in patients from 12 months of age with ALGS and received orphan exclusivity for ALGS.

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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