



PRESS RELEASE

## Ipsen completes acquisition of Albireo, expanding the scope of its Rare Disease portfolio

- Bringing medicines to patients with rare liver disease, a growth opportunity for Ipsen
- Lead asset Bylvay® (odevixibat) is the first approved treatment in progressive familial intrahepatic cholestasis, with two additional investigational indications in rare, pediatric liver diseases
- Acquisition adds novel bile-acid modulators and an innovative pipeline to the existing rare liver portfolio

**PARIS, FRANCE, 3 March 2023** – Ipsen (Euronext: IPN; ADR: IPSEY) today announced it has completed the acquisition of Albireo Pharma, Inc., a leading innovator in bile-acid modulators to treat rare liver conditions. The acquisition enriches Ipsen’s Rare Disease portfolio, with promising therapeutics for pediatric and adult rare cholestatic-liver diseases, innovative pipeline potential, as well as scientific and commercial capabilities. Pursuant to the transaction, Ipsen acquires all the issued and outstanding shares at a price of \$42.00 per share in cash plus one non-transferable contingent value right (CVR) of \$10.00 per share.

“The acquisition of Albireo will greatly strengthen our portfolio in rare diseases,” said David Loew, Chief Executive Officer of Ipsen. “I am excited to welcome new colleagues to Ipsen, who led the innovation on the development of novel bile acid modulators, like Bylvay, to treat rare liver diseases in children and adults. With Ipsen’s global presence, together we will be able to bring the full potential of the approved medicines to patients around the world.”

Lead medicine, Bylvay, is a potent once-daily ileal bile acid transport inhibitor (IBATi) that received regulatory approvals in 2021 in the U.S. for the treatment of pruritus in patients three months of age and older with progressive familial intrahepatic cholestasis (PFIC)<sup>1</sup> and in the E.U. for the treatment of PFIC in patients aged six months or older.<sup>2</sup>

In addition to the lead indication, Bylvay was accepted for Priority Review by the U.S. FDA for pediatric and adult Alagille syndrome (ALGS) in February 2023 with a Prescription Drug User Fee Act (PDUFA) action date of June 15, 2023. A variation seeking authorization for ALGS was also submitted to the EMA in 2022, which has been validated for review. In a third indication, the rare pediatric cholestatic liver disease, biliary atresia (BA), Bylvay is in late-stage development with the Phase III BOLD (Biliary atresia and the use of Odevixibat in treating Liver Disease) trial. This is the first, prospective, double-blind clinical trial in this patient population. Bylvay has orphan exclusivity for the approved indications in PFIC in the U.S. and E.U., and orphan drug designations have been granted in both ALGS and BA indications in the U.S. and E.U.

As part of the transaction, Ipsen has also acquired A3907 and A2342, two clinical-stage assets in Albireo’s pipeline. A3907 is a novel oral systemic apical sodium-dependent bile-acid transporter inhibitor currently in Phase II clinical development for primary sclerosing cholangitis (PSC).<sup>3</sup> A2342 is an oral systemic sodium-taurocholate co-transporting peptide (NTCP) inhibitor being evaluated for viral and cholestatic diseases in a Phase I trial.

As of 2 March 2023, close of business, Albireo’s common stock will cease to be traded on the NASDAQ Capital Market and will be subsequently deregistered.

## ENDS

### **About Bylvay® (odevixibat)**

Bylvay is a potent, non-systemic ileal bile-acid transport inhibitor (IBATi). It is approved in the U.S. for the treatment of pruritus in patients three months of age and older with PFIC<sup>1</sup>, where it has orphan exclusivity. Bylvay was launched 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.<sup>2</sup> 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 E.U. prescribing information here: [Bylvay, INN-odevixibat \(europa.eu\)](https://www.euro.ema.europa.eu/medicines/humans/CTX/Bylvay)

View full U.S. prescribing information here: [label \(fda.gov\)](https://www.fda.gov/oc/odp/odevixibat)

### **About BOLD**

BOLD (NCT04336722) is a double-blind, randomized, placebo-controlled trial to evaluate the efficacy and safety of Bylvay (odevixibat) in children who have biliary atresia and have undergone a Kasai procedure before age three months. Children in the treatment arm receive Bylvay 120 µg/kg orally once daily for 24 months. The primary efficacy endpoint is improvement in the proportion of patients who are alive and have not undergone a liver transplant after two years of treatment compared to placebo, and secondary outcome measures include time to onset of any sentinel events, total bilirubin levels and sBA levels.

### **About PFIC**

PFIC is a spectrum<sup>4-7</sup> of autosomal recessive genetic disorders in which cholestasis may lead to end-stage liver disease.<sup>8</sup> The estimated global incidence of PFIC is 1 in 100,000 live births.<sup>8</sup> Currently in the U.S., it is estimated that there are 500 PFIC patients who may be eligible for IBATi treatment. Subtypes PFIC1, PFIC2 and PFIC3 are the most common.<sup>8</sup> In addition, other rare forms of PFIC exist with varying degrees of cholestasis.<sup>9</sup> Patients with PFIC have impaired bile flow, or cholestasis, and the resulting bile build-up in liver cells causes liver disease and symptoms. The most debilitating symptom of PFIC is pruritus (itching), which may be so severe that it leads to skin mutilation, loss of sleep, irritability, poor attention and impaired school performance.<sup>7</sup> Up to 80% of PFIC patients suffer from severe pruritus, associated with abrasions, skin mutilation, hemorrhage or scarring.<sup>10</sup>

### **About 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.<sup>11</sup> Approximately 95% of patients with the condition present with chronic cholestasis, usually within the first three months of life and as many as 88% also present with severe, intractable pruritus.<sup>12,13</sup> The estimated global incidence of ALGS is 3 in 100,000 live births.<sup>14</sup> Currently in the U.S., it is estimated that there are 1,300 patients who may be eligible for IBATi treatment.

### **About BA**

BA is a rare pediatric liver disease. Symptoms typically develop about two to eight weeks after birth and there are no approved pharmacological therapies. Damaged or absent bile ducts outside the liver result in bile and bile acids being trapped inside the liver, quickly resulting in cirrhosis and liver failure requiring liver transplantation. At the time of diagnosis, a hepatic portoenterostomy (HPE) called Kasai procedure is performed to create a conduit allowing biliary drainage. The rate of success in re-establishing bile flow is dependent on the age of the infant when the HPE is performed. Kasai procedure is not curative and most patients who have BA have progressive disease, with at least 80% requiring liver transplantation by age 20 years.<sup>15</sup> Of those who survive into the third decade after birth, almost all have portal hypertension or other complications of cirrhosis.<sup>16</sup> New therapies are therefore needed to delay or avoid the need for liver transplantation following Kasai procedure.<sup>17</sup> There are currently no approved pharmacological treatments for biliary atresia. There is an estimated incidence of 5-6 per 100,000 live births worldwide with

BA.<sup>18</sup> Currently in the U.S., it is estimated that there are 750 patients who may be eligible for IBATi treatment.

### **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 5,000 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](https://www.ipsen.com)

### **For further information:**

#### **Ipsen Contacts**

##### **Investors**

##### **Craig Marks**

Vice President, Investor Relations  
+44 (0)7584 349 193

##### **Media**

##### **Anna Gibbins**

Global Head of Franchise Communications,  
Rare Disease  
+44 (0)7717801900

##### **Ioana Piscociu**

Senior Manager  
Global Media Relations  
+33 6 69 09 12 96

##### **Amy Wolf**

VP, Head of Corporate Brand Strategy &  
Communications  
+41 79 576 07 23

### **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](http://ipsen.com).

- 
1. [label \(fda.gov\)](http://label.fda.gov)
  2. [Bylvay, INN-odevixibat \(europa.eu\)](http://Bylvay, INN-odevixibat (europa.eu))
  3. Safety and Tolerability of A3907 in Primary Sclerosing Cholangitis - Full Text View - ClinicalTrials.gov (last accessed 21 February 2023)
  4. Henkel S. World J Hepatol. 2019;11(5):450-463
  5. Schatz B. Hepatol Commun. 2018;2(5):504-514
  6. Aldrian D. J Clin Med. 2021;10(3):481
  7. Folmer D E. Hepatology 2009;50(5):1597-1605
  8. Davit-Spraul A. Orphanet J Rare Dis. 2009;4:1
  9. Amirneni S World J Gastroenterol. 2020;26(47):7470- 7484
  10. Baker A. Clin Res Hepatol Gastroenterol. 2019;43(1):20-36
  11. U.S. Department of Health and Human Services. Alagille syndrome- about the disease. Genetic and rare diseases information center. <https://rare-diseases.info.nih.gov/diseases/804/alagille-syndrome>
  12. Singh S P.Euroasian J Hepatogastroenterol. 2018;8(2):140-147
  13. Feldman A G. Neoreviews 2013;14 (2): e63–e73
  14. Leonard L. European Journal of Human Genetics. 2014; 22:435
  15. Lykavieris P. Hepatology. 2005;4 (2):366-371
  16. Jain V. Hepatology. 2001;73 (1); 93-98
  17. Efficacy and Safety of Odevixibat in Children with Biliary Atresia Who Have Undergone a Kasai HPE (BOLD) - Full Text View - ClinicalTrials.gov
  18. Hopkins P J Pediatr. 2017;187:253-257. doi: 10.1016/j.jpeds.2017.05.006. Epub 2017 Jun 1.