PRESS RELEASE

Ipsen's palovarotene clinical program in fibrodysplasia ossificans progressiva reaches prespecified interim analysis futility criteria

Ipsen has decided to pause dosing in the palovarotene trials

Based on encouraging therapeutic activity signals observed in preliminary post-hoc analyses and recommendations from the Independent Data Monitoring Committee, Ipsen to conduct further assessment of the complete data set and work with the regulatory authorities to determine the path forward

PARIS, France, 24 January 2020 -- Ipsen (Euronext: IPN; ADR: IPSEY) today announced the decision to pause dosing patients in the global Phase III (PVO-1A-301) study designed to evaluate the efficacy and safety of palovarotene in patients with fibrodysplasia ossificans progressiva (FOP), as well as the ongoing Phase II (PVO-1A-202/204) extension studies. In both the Phase III and Phase II extension studies, palovarotene is dosed both chronically (daily) and episodically (during flare-ups). The decision to pause dosing patients in the trial is based on results of a futility analysis reviewed by the Independent Data Monitoring Committee (IDMC) as part of the prespecified interim analysis. The results of a futility analysis indicated that the Phase III FOP trial was unlikely to meet its primary efficacy endpoint (annualized change in new HO volume as compared with Natural History Study[i]) upon completion.

Despite the results of the prespecified interim analysis, signals of encouraging therapeutic activity were observed in preliminary post-hoc analyses of the Phase III trial and shared with and acknowledged by the IDMC which is recommending not to discontinue the study. In its recommendations, the IDMC notes highly disparate results precluding a confident conclusion about futility. The IDMC also points out that the protocol-prespecified model may have negatively affected the efficacy analysis and shifted the statistical conclusion from significant therapeutic benefit to showing futility of the treatment. The FDA partial clinical hold for the pediatric population under the age of 14 for FOP and multiple osteochondromas (MO) issued on 4 December 2019, remains in effect.

Ipsen will pause dosing patients in the trials and conduct further assessment of the complete data set. Based on the IDMC's observations and recommendations, Ipsen will discuss these findings with regulatory authorities to determine the path forward for the palovarotene program in FOP. Ipsen will collaborate and consult with the patients, investigators, ethics committees and regulatory authorities to define next steps for the program, in the best interests of patients, whilst ensuring consent of all parties involved.

"While the study has met prespecified statistical futility, we are encouraged by the results observed in the preliminary post-hoc analyses and look forward to discussing these with regulators as quickly as possible to determine the next steps for the palovarotene program," said Aymeric Le Chatelier, Chief Executive Officer at Ipsen. "We gratefully acknowledge the ongoing support and trust from patients, their families and the healthcare professionals involved in these trials. We are deeply committed to drug development in the area of rare and ultra-rare diseases where there are multiple high unmet medical needs and often a limited understanding of the disease itself."

Ipsen is currently assessing the financial implications of these developments, including the financial outlook for 2022, and will present in February updated views together with Full Year 2019 results.

About palovarotene
Palovarotene is a RARγ agonist being developed as a potential treatment for patients with ultra-rare and debilitating bone diseases, including fibrodysplasia ossificans progressiva (FOP) and multiple osteochondromas (MO), as well as other conditions including dry eye disease. Palovarotene, which had rare pediatric disease and breakthrough therapy designations for the treatment of an ultra-rare bone disorder, was acquired by Ipsen through the acquisition in April 2019 of Clementia Pharmaceuticals.

About Ipsen

Ipsen is a global specialty-driven biopharmaceutical group focused on innovation and Specialty Care. The Group develops and commercializes innovative medicines in three key therapeutic areas: Oncology, Neuroscience and Rare Diseases. Its commitment to oncology is exemplified through its growing portfolio of key therapies for prostate cancer, neuroendocrine tumors, renal cell carcinoma and pancreatic cancer. Ipsen also has a well-established Consumer Healthcare business. With total sales over €2.2 billion in 2018, Ipsen sells more than 20 drugs in over 115 countries, with a direct commercial presence in more than 30 countries. Ipsen's R&D is focused on its innovative and differentiated technological platforms located in the heart of the leading biotechnological and life sciences hubs (Paris-Saclay, France; Oxford, UK; Cambridge, US). The Group has about 5,800 employees worldwide. Ipsen is listed in Paris (Euronext: IPN) and in the United States through a Sponsored Level I American Depositary Receipt program (ADR: IPSEY). For more information on Ipsen, visit www.ipsen.com.

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The forward-looking statements, objectives and targets contained herein are based on the Group'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 the Group'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 the Group'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 the Group. 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 product 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. The Group must face or might face competition from generic products 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 the Group may fail to achieve its objectives and be forced to abandon its efforts with regards to a product in which it has invested significant sums. Therefore, the Group cannot be certain that favorable results obtained during pre-clinical 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 product concerned. There can be no guarantees a product will receive the necessary regulatory approvals or that the product 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 health care legislation; global trends toward health care cost containment; technological advances, new products and patents attained by competitors; challenges inherent in new product development, including obtaining regulatory approval; the Group'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 the Group's patents and other protections for innovative products; and the exposure to litigation, including patent litigation, and/or regulatory actions. The Group also depends on third parties to develop and market some of its products which could potentially generate substantial royalties; these partners could behave in such ways which could cause damage to the Group's activities and financial results. The Group 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 the Group's partners could generate lower revenues than expected. Such situations could have a negative impact on the Group's business, financial position or performance. The Group 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. The Group'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 the Group's 2018 Registration Document available on its website (www.ipsen.com).

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[i] *Prospective Natural History Study (NHS), the first multi-center, non-interventional, two-part longitudinal study designed to measure disease progression over three years in patients with FOP.