PRESS RELEASE

Ipsen announces positive outcome of FDA Advisory Committee on investigational palovarotene for fibrodysplasia ossificans progressiva

- Advisory committee voted 10 for and 4 against that evidence from the Phase III MOVE study show palovarotene is an effective treatment in patients with the ultra-rare bone disease fibrodysplasia ossificans progressiva (FOP)
- Advisory committee further voted 11 for and 3 against on the benefits of palovarotene outweighing the risks, for the treatment of patients with FOP
- FDA PDUFA action date is anticipated by August 16, 2023

PARIS, FRANCE, 29 June 2023 – Ipsen (Euronext: IPN; ADR: IPSEY) announced today that the U.S. Food and Drug Administration's (FDA) Endocrinologic and Metabolic Drugs Advisory Committee (EMDAC) voted in favor of investigational palovarotene as an effective treatment, with a positive risk-benefit profile, for people living with the ultra-rare bone disease, fibrodysplasia ossificans progressiva (FOP). The FDA is currently reviewing the New Drug Application (NDA) for palovarotene with a decision anticipated by August 16, 2023. If approved, palovarotene will be the first treatment in the U.S. for FOP.

"We are pleased with the outcome today and believe that the vote of the FDA advisory committee conveys the potential of palovarotene in helping manage the severe impacts of FOP. For people living with this ultra-rare bone disease, mobility is severely restricted and they experience a significant loss of function, all of which completely changes the course of their lives and can shorten life-expectancy," said Howard Mayer, Executive Vice President and Head of Research and Development for Ipsen. "We are very grateful to the patients and healthcare professionals who have participated in our clinical trials and to the individuals who selflessly shared their very personal experiences of living with FOP during the advisory committee. We will continue to work closely with the FDA on the next steps."

The advisory committee voted 10 for and 4 against that evidence from the Phase III MOVE study show palovarotene is an effective treatment in patients with the ultra-rare bone disease fibrodysplasia ossificans progressiva (FOP). The committee further voted 11 for and 3 against on the benefits of palovarotene outweighing the risks, for the treatment of patients with FOP.

FOP is an ultra-rare disease that causes permanent and continuous bone growth in soft and connective tissues like muscles, tendons and ligaments, also known as heterotopic ossification or HO.[1] FOP impacts the lives of an estimated 400 people in the U.S. and 900 people globally.[2,3] As the disease continuously progresses with flare-up episodes causing rapid bone growth, FOP severely restricts mobility and function.[3] Most people living with FOP inevitably lose the ability to eat and drink on their own, can not provide self-care or use the restroom themselves, and are unable to maintain employment.[4] By the age of 30, most people with FOP require a wheelchair and full-time caregiver assistance.[2] Without disease-modifying treatments, current management is limited to palliative care, and ultimately, FOP shortens the median life expectancy to 56 years as untimely death is caused by bone formation around the ribcage leading to breathing problems and cardiorespiratory failure.[2]

The advisory committee's recommendation is based on its review of the efficacy and safety data package for palovarotene, that included results from the Phase III MOVE trial, the first and largest multicenter,

open-label trial in adult and pediatric patients, which demonstrated a clinically meaningful reduction in new abnormal bone formation (HO) and a well-characterized safety profile.[5]

Palovarotene; a potential treatment for FOP

Palovarotene is an investigational oral medicine that selectively targets the retinoic-acid receptor gamma (RARγ), which is an important regulator of skeletal development and ectopic bone in the retinoid signaling pathway. Palovarotene is designed to mediate the interactions between the receptors, growth factors and proteins within the retinoid signaling pathway to reduce new abnormal bone formation (HO). Palovarotene received Orphan Drug and Breakthrough Therapy Designations from the U.S. Food and Drug Administration (FDA) for the potential treatment of FOP and was granted Priority Review. Palovarotene is also under review with a number of regulatory authorities. Palovarotene is currently authorized for use in appropriate patients in Canada and United Arab Emirates where it is marketed as Sohonos™ (palovarotene capsules).[6]

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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

For further information:

Contacts

Investors

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

Nicolas Bogler
Investor Relations Manager
+33 6 52 19 98 92

Media

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

Rachel Reiff
U.S. Head of Franchise Communications
+1 908 616 1680

Anna Gibbins
Global Head of Franchise Communications, Rare Disease
+44 7717801900

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

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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1 Kaplan FS, et al. The medical management of fibrodysplasia ossificans progressiva: current treatment considerations. Proc Intl Clin Council FOP 1:1-111, 2019.
2 Liljesthröm M, Pignolo RJ, Kaplan FS. Epidemiology of the Global Fibrodysplasia Ossificans Progressiva (FOP) Community. J Rare Dis Res Treat. (2020) 5(2): 31-36
3 Pignolo, RJ et al. Bone. 2020; 134:115274.
4 Al Mukaddam M, et al. Val Health 2022;25:S273 (POSA427)
5 Pignolo RJ, Hsiao E, Al Mukaddam M et al. Reduction of New HO in the Open-Label, Phase 3 MOVE Trial of Palovarotene for Fibrodysplasia Ossificans Progressiva (FOP). J Bone Miner Res. 2022.
6 Government of Canada, Notice Multiple Additions to the Prescription Drug List (PDL). Viewed 30 November 2022, <https://www.canada.ca/en/health-canada/services/drugs-health-products/drug-products/prescription-drug-list/notices-changes/multiple-additions-2022-01-24.html>.