PRESS RELEASE

Ipsen announces European Commission decision on palovarotene for the treatment of FOP

- European Commission determines not to grant marketing authorization approval for palovarotene for fibrodysplasia ossificans progressiva (FOP)
- Decision follows negative opinion by the Committee for Medicinal Products for Human Use, in May
- No currently approved options available in the E.U. for the treatment of FOP, an ultra-rare condition, that continuously and permanently causes abnormal bone formation, leading to progressive mobility loss and shortened life expectancy
- Ipsen to continue seeking regulatory approvals in other countries and regions

PARIS, FRANCE, 19 July 2023 – Ipsen (Euronext: IPN; ADR: IPSEY) announced today that the European Commission has followed guidance provided by the Committee for Medicinal Products for Human Use (CHMP) in May this year, and has not granted marketing authorization for palovarotene, an investigational treatment for fibrodysplasia ossificans progressiva (FOP). Palovarotene is the first treatment to be submitted anywhere in the world for regulatory approval for FOP, an ultra-rare disease with approximately 900 known cases worldwide.

“We worked tirelessly to bring a greatly needed treatment option to patients living with FOP in the E.U.,” said Howard Mayer, Executive Vice President and Head of Research and Development for Ipsen. “We believe that our clinical data provide evidence supporting the effect of palovarotene on the reduction of new, abnormal bone formation, known as heterotopic ossification, which characterizes the disease. We are therefore disappointed that the European Commission decided not to approve this treatment for patients with FOP in Europe. We have learned through this experience, and we have been motivated by the support of the FOP community, including those living with the condition and the doctors and healthcare providers managing their care. This has encouraged us as we continue to progress with other regulatory submissions.”

Palovarotene was studied in a comprehensive clinical program over 15 years. This included MOVE, the first and largest Phase III clinical trial for FOP, a condition that causes abnormal bone growth. The average age of diagnosis for FOP is five years old and the average life expectancy is 56 years old. FOP is a chronic and progressive condition, where flare-ups occur that can lead to the development of new, abnormal bone formulation, accumulating outside of the skeleton in muscles, joints, and other areas of the body. As a result, most people living with FOP eventually lose the ability to eat and drink on their own. By the age of 30 years old, many will need a wheelchair to get around and full-time care. Life expectancy is shortened, as untimely death can be caused by bone formation around the ribcage, leading to breathing problems and cardiorespiratory failure.

“It is devastating to hear that the wait for an innovative treatment for people living with FOP, and their families and caregivers will continue, as this negative decision from the European Commission means that this treatment will not be made available to patients.” said Dr. Genevieve Baujat, Clinical Geneticist Consultant at Necker-Enfants Malades Hospital, Paris, France. “Many of the clinicians that manage patients with this disease in Europe will have participated in the MOVE clinical trial and have seen the potential for palovarotene.”

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About palovarotene

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. 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. It remains under FDA review, with a Prescription Drug User Fee Act goal date of 16 August 2023. Palovarotene was also granted orphan medicine designation by the European Medicines Agency (EMA). Palovarotene is in review processes with a number of regulatory authorities including the FDA and the EMA. Palovarotene is currently authorized for use in appropriate patients only in Canada and provisionally in the U.A.E. where it is marketed as Sohonos™ (palovarotene capsules).

About the MOVE trial

MOVE (NCT03312634) was a Phase III, multicenter, single-arm, open-label trial to assess the efficacy and safety of palovarotene. 107 study participants with FOP received oral palovarotene as a chronic (5mg once daily) and episodic (20mg once daily for 4 weeks, followed by 10mg for ≥8 weeks for flare-ups and trauma) regimen. The primary endpoint was annualized change in new HO volume measured by low-dose whole-body computed tomography. Efficacy data from participants enrolled in MOVE were compared with data from FOP Natural History Study (NHS) participants untreated beyond standard of care; individuals ≤65 years of age with clinically diagnosed FOP and a verified ACVR1R206H pathogenic variant were eligible for inclusion in the NHS.

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

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Ipsen's forward-looking statements

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