IPSÉN’S PALOVAROTENE CLINICAL PROGRAM IN FIBRODYSPLASIA OSSIFICANS PROGRESSIVA REACHES PRESPECIFIED INTERIM ANALYSIS FUTILITY CRITERIA

IPSÉN 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, IPSÉN 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 -- IPSÉN (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) 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.

IPSÉN WILL PAUSE DOSING PATIENTS IN THE TRIALS AND CONDUCT FURTHER ASSESSMENT OF THE COMPLETE DATA SET. BASED ON THE IDMC’S OBSERVATIONS AND RECOMMENDATIONS, IPSÉN WILL DISCUSS THESE FINDINGS WITH REGULATORY AUTHORITIES TO DETERMINE THE PATH FORWARD FOR THE PALOVAROTENE PROGRAM IN FOP. IPSÉN 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 IPSÉN. “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.”

IPSÉN 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 IPSÉN 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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*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.