PRESS RELEASE

Ipsen to present new insights at ASBMR for potential treatment of ultra-rare disease fibrodysplasia ossificans progressiva (FOP), including global Phase III MOVE trial results

- Seven presentations represent Ipsen's largest presence to date at ASBMR, the world's leading scientific organization for bone health research

- Ipsen's data demonstrate potential advances in understanding fibrodysplasia ossificans progressiva (FOP), including key learnings related to study design, natural history, biomarkers, investigational therapeutic options, and disease-specific patient-reported outcome measures

PARIS, FRANCE, 10 September 2020 – Ipsen (Euronext: IPN: ADR: IPSEY) today announced that it will share data from the company's growing Rare Diseases Therapeutic Area portfolio, with seven presentations at the American Society for Bone and Mineral Research (ASBMR) Annual Meeting (September 11-15, 2020). These include the oral presentation of Ipsen's MOVE trial (during the Novel Therapies for Rare Bone Disease session on Saturday, 12 September [11:00am - 12:15pm ET]), the first and only multicenter Phase III study in fibrodysplasia ossificans progressiva (FOP). The data will be presented by Dr Robert Pignolo, Division of Geriatric Medicine and Gerontology, Department of Internal Medicine, Mayo Clinic, and describes the trial outcomes of the oral investigational therapy palovarotene in reducing new heterotopic ossification (HO) volume in 107 pediatric and adult patients with FOP.[1] The MOVE efficacy results were compared with data from untreated patients from Ipsen's Natural History Study (NHS).[2] Safety outcomes from the MOVE trial will also be presented.

"Our passion and commitment to understanding rare diseases has driven this research forward and we're proud to present these data at the ASBMR 2020 Annual Meeting," said Howard Mayer, M.D., Executive Vice President and Head of Research and Development at Ipsen. "Collaboration is critical with rare disease research and development and we look forward to continuing to work with key thought leaders, clinicians, the patient advocacy community, and regulatory authorities as we build on our research and develop potential therapeutic agents in rare diseases, including for patients with FOP."

Additionally, four posters reporting data from the NHS will be presented. The NHS is the largest FOP study of this kind worldwide and is the first global, multicenter, longitudinal study designed to measure disease progression over three years.[2] Findings from the NHS reinforce that measuring HO is a viable way to monitor changes in FOP and to assess a potential treatment effect over this time period.

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Overview of all Ipsen presentations at ASBMR 2020 Annual Meeting:

Medicine/disease	Abstract number/title	Study authors
Palovarotene/FOP	Palovarotene (PVO) for Fibrodysplasia Ossificans Progressiva (FOP): Data from the Phase III MOVE Trial	Robert J. Pignolo, Mona Al Mukaddam, Geneviève Baujat, Staffan K. Berglund, Angela M. Cheung, Carmen De Cunto, Patricia Delai, Maja Di Rocco, Nobuhiko Haga, Edward C. Hsiao, Peter Kannu, Richard Keen, Edna E. Mancilla, Donna R. Grogan, Rose Marino, Andrew Strahs, Frederick S. Kaplan
Palovarotene/FOP	Measuring outcomes in ultra-rare bone diseases: Methodology of the palovarotene fibrodysplasia ossificans progressiva (FOP)	Robert J. Pignolo, Geneviève Baujat, Matthew A. Brown, Carmen De Cunto, Maja Di Rocco, Edward C. Hsiao, Richard Keen, Mona Al Mukaddam, Andrew Strahs, Donna R. Grogan, Rose Marino, Frederick S.

	clinical development program	Kaplan
FOP	Medications used by individuals with Fibrodysplasia Ossificans Progressiva (FOP): Data from a global natural history study	Richard Keen, Mona Al Mukaddam, Geneviève Baujat, Carmen De Cunto, Edward C. Hsiao, Robert J. Pignolo, Kathleen Harnett, Rose Marino, Frederick S. Kaplan
FOP	Longitudinal and flare-up-specific biomarkers in fibrodysplasia ossificans progressiva (FOP): Data from a global natural history study	Robert J. Pignolo, Mona Al Mukaddam, Geneviève Baujat, Carmen De Cunto, Edward C. Hsiao, Richard Keen, Kathleen Harnett, Rose Marino, Frederick S. Kaplan
FOP	Use of assistive devices and adaptations by individuals with fibrodysplasia ossificans progressiva (FOP): Data from a global natural history study	Edward C. Hsiao, Mona Al Mukaddam, Geneviève Baujat, Carmen De Cunto, Richard Keen, Robert J. Pignolo, Kathleen Harnett, Rose Marino, Frederick S. Kaplan
FOP	A global natural history study of fibrodysplasia ossificans progressiva (FOP): 12-month outcomes	Mona Al Mukaddam, Robert J. Pignolo, Geneviève Baujat, Matthew A. Brown, Carmen De Cunto, Maja Di Rocco, Edward C. Hsiao, Richard Keen, Kim-Hanh Le Quan Sang, Andrew Strahs, Rose Marino, Frederick S. Kaplan
FOP	Validity and reliability of the fibrodysplasia ossificans progressiva physical function questionnaire (FOP-PFQ), a patient-reported, disease-specific measure	Robert J. Pignolo, Miriam Kimel, John Whalen, Ariane Kawata, Dennis Revicki, Rose Marino, Frederick S. Kaplan

About palovarotene
Palovarotene is an oral investigational, selective RARγ agonist being developed as a potential treatment for patients with the debilitating ultra-rare, genetic disorder fibrodysplasia ossificans progressiva (FOP). Palovarotene, which had rare pediatric disease and breakthrough therapy designations for the treatment of FOP, was acquired by Ipsen through the acquisition of Clementia Pharmaceuticals in April 2019.

About fibrodysplasia ossificans progressiva (FOP)
Fibrodysplasia ossificans progressiva (FOP) is an ultra-rare, genetic disorder characterized by bone that forms outside the normal skeleton, in muscles, tendons or soft tissue.[3] FOP is among the rarest of human diseases, and while there are approximately 1,000 described cases globally, the reported prevalence of FOP is estimated at approximately 1.36 per million individuals.[4,5]

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. Ipsen also has a well-established Consumer Healthcare business. With total sales over €2.5 billion in 2019, 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

Ipsen's Forward Looking Statement
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, and the outcome of this study or other studies. 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 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 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 6 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 2019 Universal Registration Document available on its website (www.ipsen.com).

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2. Al Mukaddam M et al. A Natural History Study of Fibrodysplasia Ossificans Progressiva (FOP): 12-Month Outcomes. J Endocr Soc. 2020;4 (Supplement 1):OR29-05
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