

Ipsen and Blueprint Medicines announce exclusive global license agreement to develop and commercialize BLU-782 for the treatment of fibrodysplasia ossificans progressiva (FOP)

-- Expands Ipsen's Rare Diseases portfolio to include BLU-782, a highly selective investigational ALK2 inhibitor for the treatment of FOP --

-- Accelerates global development of BLU-782 through Ipsen's clinical expertise in rare diseases and global infrastructure --

-- Blueprint Medicines is eligible to receive up to \$535 million, including a \$25 million upfront payment and up to \$510 million in potential development, regulatory and sales-based milestones and other payments, plus tiered percentage royalties --

PARIS, France and CAMBRIDGE, Mass., USA, OCTOBER 16, 2019 /PRNewswire/ -- Ipsen (Euronext: IPN; ADR: IPSEY) and Blueprint Medicines Corporation (NASDAQ: BPMC) today announced Ipsen, through its subsidiary Clementia Pharmaceuticals, and Blueprint Medicines have entered into an exclusive, worldwide license agreement for the development and commercialization of BLU-782, an oral, highly selective investigational ALK2 inhibitor being developed for the treatment of fibrodysplasia ossificans progressiva (FOP).

The agreement enhances Ipsen's Rare Diseases portfolio and advances Blueprint Medicines' goal of rapidly and efficiently developing BLU-782 as a potential treatment for patients with FOP. Ipsen has demonstrated its commitment to leadership in this complex ultra-rare genetic disorder through the ongoing late-stage clinical development of palovarotene, an investigational retinoic acid receptor gamma (RAR γ) agonist. With the addition of BLU-782, which recently completed dosing in a Phase 1 study in healthy volunteers, Ipsen has the potential to offer the broadest possible suite of treatment options for patients with FOP.

"Our strategy has been to build a leading Rare Diseases franchise, and through the recent acquisition of Clementia, we gained a first-in-class asset in palovarotene. Now, with the addition of Blueprint Medicines' BLU-782, we have two strong complementary drug candidates. We will continue to develop and deliver valuable treatments for patients around the world living with FOP and other rare diseases," said David Meek, CEO, Ipsen.

"We admire Ipsen's track record of successful global clinical development in this complex, ultra-rare genetic disorder and believe this expertise, combined with Ipsen's global infrastructure and commitment to transforming the treatment of FOP, will accelerate the development of BLU-782 globally," said Jeff Albers, CEO, Blueprint Medicines. *"We are inspired by the FOP community, including the patients, families, clinicians and advocacy groups we have had the fortune to work with, as we have advanced this program from an idea to BLU-782, the first investigational therapy targeting ALK2, the genetic driver of FOP, to enter clinical development. We are also grateful for the dedication and drive of our team at Blueprint Medicines whose tireless commitment has brought BLU-782 this far."*

Subject to the terms of the license agreement, Blueprint Medicines will be eligible to receive up to \$535 million in upfront, milestone and other payments, including an upfront cash payment of \$25 million and up to \$510 million in potential milestone payments related to specified development, regulatory and sales-based milestones for licensed products in up to two indications, including FOP. In addition, Ipsen will pay Blueprint Medicines tiered percentage royalties ranging from the low- to mid-teens on worldwide aggregate annual net sales of licensed products, subject to adjustment in specified circumstances under the license agreement.

About BLU-782

BLU-782 was designed by Blueprint Medicines to selectively target mutant ALK2, the underlying cause of FOP, using Blueprint Medicines' proprietary scientific platform. Blueprint Medicines recently completed dosing in a Phase 1 clinical trial of BLU-782 in healthy volunteers and reported preliminary data at the American Society

of Bone and Mineral Research Annual Meeting in September 2019, which showed that BLU-782 was well-tolerated at all doses tested. Previously reported preclinical data in a well-characterized, genetically accurate FOP model showed that BLU-782 prevented injury- and surgery-induced heterotopic ossification, reduced edema and restored healthy tissue response to muscle injury. The FDA has granted a rare pediatric disease designation, orphan drug designation and fast track designation to BLU-782.

About fibrodysplasia ossificans progressiva (FOP)

FOP is a rare, severely disabling genetic disorder characterized by progressive heterotopic ossification (HO), or the abnormal transformation of muscle, ligaments and tendons into bone. HO may be spontaneous or associated with painful episodic disease flare-ups that are usually precipitated by soft tissue injury. As the disease progresses, extra-skeletal bone increasingly restricts joints, resulting in severe disability and loss of mobility, compromised respiratory function and increased risk of early death. FOP is caused by a mutation in the gene for ALK2, which is known as ACVR1, leading to inappropriate activation of the bone morphogenetic pathway.

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 5,700 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.

About Blueprint Medicines

Blueprint Medicines is a precision therapy company striving to improve human health. With a focus on genomically defined cancers, rare diseases and cancer immunotherapy, we are developing transformational medicines rooted in our leading expertise in protein kinases, which are proven drivers of disease. Our uniquely targeted, scalable approach empowers the rapid design and development of new treatments and increases the likelihood of clinical success. We are currently advancing three investigational medicines in clinical development, along with multiple research programs. For more information, visit www.BlueprintMedicines.com and follow us on Twitter (@BlueprintMeds) and LinkedIn.

Ipsen—Cautionary Note Regarding Forward-Looking Statements

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).

Blueprint Medicines—Cautionary Note Regarding Forward-Looking Statements

This press release contains forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995, as amended, including, without limitation, statements regarding Ipsen's plan to develop and commercialize BLU-782 or any other licensed products under the license agreement; potential payments under the license agreement, including the upfront payment and any milestone or royalty payments; potential benefits of the license agreement between Blueprint Medicines and Ipsen; the potential benefits of BLU-782 or any other licensed product in treating patients, including patients with FOP; and Blueprint Medicines' strategy, goals and anticipated milestones, business plans and focus. The words "may," "will," "could," "would," "should," "expect," "plan," "anticipate," "intend," "believe," "estimate," "predict," "project," "potential," "continue," "target" and similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words. Any forward-looking statements in this press release are based on management's current expectations and beliefs and are subject to a number of risks, uncertainties and important factors that may cause actual events or results to differ materially from those expressed or implied by any forward-looking statements contained in this press release, including, without limitation, risks and uncertainties related to the delay of any current or planned clinical trials or the development of Blueprint Medicines' drug candidates or the licensed products, including BLU-782; the ability of the Blueprint Medicines and Ipsen to terminate the license agreement under specified circumstances; preclinical and clinical results for BLU-782 that may not support further development of such drug candidate, including expectations that such results may be predictive of the results in future clinical trials; actions of regulatory agencies, which may affect the initiation, timing and progress of clinical trials or the regulatory pathway for the licensed products; and Ipsen's ability to successfully develop and commercialize the licensed products. These and other risks and uncertainties are described in greater detail in the section entitled "Risk Factors" in Blueprint Medicines' filings with the Securities and Exchange Commission (SEC), including Blueprint Medicines' most recent Quarterly Report on Form 10-Q and any other filings that Blueprint Medicines has made or may make with the SEC in the future. Any forward-looking statements contained in this press release represent Blueprint Medicines' views only as of the date hereof and should not be relied upon as representing its views as of any subsequent date. Except as required by law, Blueprint Medicines explicitly disclaims any obligation to update any forward-looking statements.

Ipsen investor contact

Financial Community
Eugenia Litz
Vice President, Investor Relations
+44 (0) 1753 627721
eugenia.litz@ipsen.com

Myriam Koutchinsky
Investor Relations Manager
+33 (0) 1 58 33 51 04
Myriam.koutchinsky@ipsen.com

Ipsen media contact

Christian Marcoux, M.Sc.
SVP, Global Communications
+33 (0) 1 58 33 67 94
christian.marcoux@ipsen.com

David Caruba
Global Communications
+1 857-998-7036
david.caruba@ipsen.com

Fanny Allaire
Global Communications
+ 33 (0) 1 58 33 58 96
fanny.allaire@ipsen.com

Blueprint Medicines investor contact

Kristin Hodous
Senior Manager, Investor Relations
617-714-6674
ir@blueprintmedicines.com

Blueprint Medicines media contact

Jim Baker
Vice President, Corporate Affairs
617-844-8236
media@blueprintmedicines.com