

# Inventiva Announces Enrollment of First Patient into Its Odiparcil Phase IIa Trial (iMProveS) for MPS VI

- ► Trial to enroll 24 patients at 2 clinical trial sites
- Results expected during the first semester of 2019

**Daix (France), January 3, 2018** — Inventiva, a biopharmaceutical company developing innovative therapies, particularly to treat fibrosis, today announced the enrollment of the first patient in its Phase IIa iMProveS (improve MPS treatment) trial targeting mucopolysaccharidosis VI patients (MPS VI).

"Odiparcil is the first new treatment in development for MPS VI patients in over a decade and is a potential significant step forward for patients," said Jean-Louis Abitbol, M.D., M.Sc., Inventiva's Chief Medical Officer and Head of Development, "Current enzyme replacement therapy requires weekly infusions, therefore an orally available therapeutic such as odiparcil would greatly increase the quality of life of patients. More importantly, thanks to its optimal distribution in the body, odiparcil has shown efficacy in tissues and organs where current enzyme replacement therapy is not effective. Overall we believe odiparcil could meaningfully improve the lives of these patients and become the standard of care in MPS VI."

The iMProveS clinical study is a 26-week study designed to demonstrate the safety, tolerability, and efficacy of odiparcil in 18 adult MPS VI patients receiving enzyme replacement therapy (ERT). The study also has an open-label arm with 6 patients currently not on ERT. If positive, the Company plans to pursue a pivotal Phase III study of odiparcil in patients with MPS VI. Odiparcil has received orphan drug designation for MPS VI in the United States and Europe. Results from the Phase IIa study are expected during the first semester of 2019.

### **About MPS VI**

MPS VI (Maroteaux-Lamy syndrome), is a rare, pediatric, genetic, degenerative disease characterized by the abnormal functioning of the enzyme N-acetylgalactosamine 4-sulphatase (arylsulphatase B; ASB) leading to the accumulation of dermatan sulfate and chondroitin sulfate in the cells, tissues and organs. Patients have coarse faces, short stature, corneal clouding, hearing loss, dysostosis multiplex, hepatosplenomegaly, cardiac valve disease and reduced pulmonary function without intellectual deficit. As with other MPS, the time of onset, rate of progression and extent of the disease may vary between the affected individuals. The life expectancy of MPS VI patients, if untreated, is approximately 20 years in patients with severe forms of the disease, or longer in patients with less severe forms. The prevalence of MPS VI is estimated to be 1 in 225,000 live births and varies between countries.

There is no cure for MPS VI and current treatment options such as enzyme replacement therapy (ERT) or hematopoietic stem cell transplant (HSCT) leave the patients with high unmet medical needs.

# About Inventiva: www.inventivapharma.com

Inventiva is a biopharmaceutical company specialized in the development of drugs interacting with nuclear receptors, transcription factors and epigenetic modulators. Inventiva's research engine opens up novel breakthrough therapies against fibrotic diseases, cancers and orphan diseases with substantial unmet medical needs.

Lanifibranor, its lead product, is an anti-fibrotic treatment with a strong action mechanism permitting the activation of all three alpha, gamma and delta PPARs (peroxisome proliferator-activated receptors), which play key roles in controlling the



fibrotic process. Its anti-fibrotic action targets two initial indications with substantial unmet medical need: NASH, a severe and increasingly prevalent liver disease already affecting over 30 million people in the United States, and systemic sclerosis, a disease with a very high mortality rate and for which there is no approved treatment to date.

Inventiva is also developing in parallel, a second clinical product, Odiparcil (formerly IVA336), which is a treatment for three different forms of mucopolysaccharidosis: MPS I or Hurler/Scheie syndromes, MPS II or Hunter syndrome and MPS VI also known as Maroteaux-Lamy syndrome. Inventiva is also developing a preclinical stage oncology portfolio.

Inventiva benefits from partnerships with world-leading research entities such as the Institut Curie. Two strategic R&D partnerships have also been established with AbbVie and Boehringer Ingelheim, making Inventiva eligible for preclinical, clinical, regulatory and commercial milestone payments, in addition to royalties on the products resulting from the partnerships.

Inventiva employs over 100 highly qualified employees and owns state-of-the-art R&D facilities near Dijon, acquired from the international pharmaceutical group Abbott. The Company owns, a proprietary chemical library of over 240,000 molecules as well as integrated biology, chemistry, ADME and pharmacology platforms.

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Please refer to the « Document de référence » filed with the Autorité des Marchés Financiers on April 26, 2017 under n° R.17-025 for additional information in relation to such factors, risks and uncertainties.

Inventiva has no intention and is under no obligation to update or review the forward-looking statements referred to above. Consequently Inventiva accepts no liability for any consequences arising from the use of any of the above statements.