

Inventiva's Odiparcil Awarded Orphan Drug Designation for the Treatment of MPS VI by the European Medicines Agency

- ▶ FDA Orphan Drug Designation received earlier this month
- ▶ EMA and FDA Orphan designations validate odiparcil potential to improve treatment options for MPS VI patients
- ▶ iMProveS phase IIa study in MPS VI patients on track to begin recruitment by year-end 2017

Daix (France), August 29, 2017 at 07:30am CEST – Inventiva, a biopharmaceutical company developing innovative therapies, particularly in fibrosis, today announced that the European Medicines Agency (EMA) has granted Orphan Drug Designation to odiparcil (formerly IVA336) for the treatment of MPS VI.

"This decision could accelerate the availability of a much needed new treatment for MPS VI patients," said Christine Lavery, President of the UK MPS Society.

"Odiparcil has the potential to become a breakthrough treatment for patients affected with MPS VI and I am looking forward to the upcoming phase IIa clinical trial which could prove the efficacy of odiparcil as a stand-alone treatment," added Professor Chris Hendriksz, of FYMCA Medical Ltd. and University of Pretoria, South Africa.

"We recently received U.S. orphan drug status and with this new EU designation we continue delivering on our regulatory strategy for odiparcil. Clearly the recent preclinical data we generated showing that odiparcil is active in organs where marketed enzyme replacement therapies have limited or no efficacy, has been instrumental in convincing regulators to grant these ODD designations. These designations confirm that regulatory agencies share our view that odiparcil could improve MPS VI current treatment options," concluded Pierre Broqua, Chief Scientific Officer and Co-Founder of Inventiva.

MPS VI (Maroteaux-Lamy syndrome), is a rare pediatric genetic degenerative disease with a prevalence estimated to be 1 in 225,000 live births. 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.

Odiparcil, an orally available therapy, is being developed as a potential therapy for MPS I, II, and VI patients. Inventiva is currently launching the Phase IIa iMProveS (improve MPS treatment) study, which is expected to enroll its first patient before year end. The iMProveS clinical study is a 26-week study designed to demonstrate the safety, tolerability, and efficacy of odiparcil in 24 adult MPS VI patients and will be conducted at two European clinical sites. If the results of this study are positive, the company plans to pursue a pivotal Phase III study of odiparcil in patients with MPS VI.

About Orphan Drug Designation

The EMA grants Orphan Drug Designation to support the development of medicines for the treatment, prevention or diagnosis of a disease that is life-threatening or chronically debilitating and that affect no more than 5 in 10,000 individuals in the European Union. Orphan drug designation allows for companies to receive development incentives, such as protocol assistance, reduced fees for regulatory activities, and up to ten years of market exclusivity in the EU upon marketing approval for the designated indication.

About MPS VI

MPS VI (Maroteaux-Lamy syndrome), is a rare pediatric genetic degenerative diseases 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.

IVA337, 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, 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 has a preclinical stage oncology portfolio.

Inventiva benefits from partnerships with world-leading research entities such as the Institut Curie. Two strategic commercial partnerships, one of which is at clinical stage, have also been developed 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.