

Odiparcil Awarded Orphan Drug Designation for the Treatment of MPS VI by the FDA

- ▶ Odiparcil therapeutic potential and significant benefit over existing therapeutic options confirmed
- ▶ With a high medical need still existing, odiparcil could significantly improve the treatment options for MPS VI patients
- ▶ First patient enrollment in a Phase IIa study expected by year-end 2017

Daix (France), August 10, 2017 at 5:45 pm CEST – Inventiva, a biopharmaceutical company developing innovative therapies, particularly in fibrosis, today announced that the FDA has granted Orphan Drug Designation to odiparcil (formerly IVA336) for the treatment of MPS VI.

MPS VI (Maroteaux-Lamy syndrome), is a rare pediatric genetic degenerative disease. The prevalence of MPS VI is 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.

“MPS are devastating diseases and odiparcil could become an important advance in providing optimal therapy for many patients struggling with lysosomal disorders,” said Professor Paul Harmatz (Children's Hospital & Research Center of Oakland, Oakland, California).

“The FDA’s decision to award Orphan Drug Designation to odiparcil in the treatment of MPS VI patients is another important milestone as it confirms that odiparcil may bring a significant therapeutic benefit over existing approved medications”, added Pierre Broqua, Chief Scientific Officer and Co-Founder of Inventiva. *“MPS VI remains a severe disease despite the availability of an enzyme replacement therapy, and this decision is a strong and encouraging signal from the FDA to move forward with our clinical program.”*

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 initiate a pivotal Phase III study in the US and in the EU with odiparcil in MPS VI patients.

About Orphan Drug Designation:

Orphan Drug Designation is granted by the FDA to novel therapeutics for diseases or conditions affecting fewer than 200,000 patients in the U.S. or greater than 200,000 patients if there is no reasonable expectation that the production cost of the drug will be covered by its sales. The designation allows the drug developer to be eligible for a seven-year period of U.S. marketing exclusivity upon approval of the drug, as well as, in some cases, tax credits for clinical research costs, the ability to apply for annual grant funding, clinical trial design assistance, and the waiver of Prescription Drug User Fee Act (PDUFA) filing fees.

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 for patients with severe forms of the disease and more for patients with less severe forms.

The prevalence of MPS VI is estimated to be 1 in 225,000 live births. It 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.