

## Inventiva to present *in vivo* data with odiparcil at the 14<sup>th</sup> *WORLDSymposium*<sup>™</sup>

Data confirms potential as first oral therapy for MPS VI patients

**Daix (France), January 31<sup>st</sup>, 2018** – Inventiva, a biopharmaceutical company developing innovative breakthrough therapies, particularly for the treatment of fibrotic diseases, today announced that Dr Eugeni V. Entchev, Head of odiparcil preclinical pharmacology program at Inventiva, will give a presentation entitled “Odiparcil is a promising substrate reduction therapy in MPS VI murine model” at the 14<sup>th</sup> *WorldSymposium*<sup>™</sup> on February 7, 2018, in San Diego, California.

After an initial closed session presentation during the MPS Society National Conference in July 2017, this will be the first public presentation of the data generated in a genetic mouse model for MPS VI (Maroteaux-Lamy syndrome) after a 6-month treatment period with two doses of odiparcil. The data demonstrate that odiparcil reduced GAG (glycosaminoglycan(s)) accumulation in the liver, kidney, spleen, heart, eye, and skin of diseased animals. Odiparcil also restored normal corneal structure in the eye and reduced thickening of the trachea and femoral growth plate cartilages. These important findings suggest that odiparcil could lead to treating eye- and cartilages-related clinical manifestations seen in MPS VI patients. Finally, mobility was improved by odiparcil in diseased animals.

**Presentation title:** “Odiparcil is a promising substrate reduction therapy in MPS VI murine model”  
**Presenter:** Dr Eugeni V. Entchev, Head of odiparcil preclinical pharmacology program, Inventiva  
**Session:** Translational Research I  
**Date/Time:** Wednesday, Feb. 7, at 10:45 a.m. PT  
**Location:** Manchester Grand Hyatt, 1 Market Place, San Diego, California, U.S.A.

### **About *WORLDSymposium*<sup>™</sup>**

The *WORLDSymposium*<sup>™</sup> is a leading annual research conference dedicated to lysosomal diseases. Since 2002, the W.O.R.L.D. (*We’re Organizing Research on Lysosomal Diseases*) meeting has grown to an international research conference that attracts over 1600 participants from more than 50 countries around the globe.

### **About odiparcil**

Odiparcil is the first new treatment in development for MPS VI in over a decade. The current standard of care is enzyme replacement therapy (ERT), which requires weekly infusions. An orally available therapeutic such as odiparcil would greatly increase the quality of life of patients. More importantly, the data generated in MPS VI mice demonstrate that odiparcil could treat clinical manifestations linked to GAG accumulation in tissues and organs where current ERT is not effective. Odiparcil is well distributed in the body even in tissues that are poorly vascularized, such as cartilages, or protected by a barrier, such as the eye. Inventiva believes odiparcil could meaningfully improve the lives of MPS VI patients, and become the new standard of care. On December 30, 2017, the first patient was enrolled in the Phase IIa iMProveS (improve MPS treatment) trial of odiparcil in MPS VI patients. Results from this study are expected in H1 2019. Odiparcil has received orphan drug designation for MPS VI in the United States and Europe.

### About MPS VI

MPS VI 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 suffer from short stature, corneal clouding, hearing loss, dysostosis multiplex, hepatosplenomegaly, cardiac valve disease and reduced pulmonary function. 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 ERT or hematopoietic stem cell transplant (HSCT) leave the patients with high unmet medical needs.

**About Inventiva:** [www.inventivapharma.com](http://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 odiparcil (formerly IVA336) 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.*