**The FDA Grants Rare Pediatric Disease Designation to Odiparcil for the Treatment of MPS VI**

* Inventiva eligible to receive Priority Review Voucher upon approval of odiparcil for the treatment of MPS VI
* Priority Review Voucher allows to reduce FDA review time from 12 to 6 months or can be transferred for prices that have ranged from $67.5 million to $350 million

**Daix (France), March 05, 2019** – Inventiva (Euronext: IVA), a clinical-stage biopharmaceutical company developing oral small molecule therapies for the treatment of diseases in the areas of fibrosis, lysosomal storage disorders and oncology, today announced that the U.S. Food and Drug Administration (FDA) has granted rare pediatric disease designation (RPDD) to odiparcil, the Company’s product candidate in development for the treatment of mucopolysaccharidosis (MPS) VI, a type of rare, progressive genetic disorder characterized by a deficiency in the lysosomal enzymes responsible for the normal degradation of glycosaminoglycans (GAGs). The designation of rare pediatric disease status confirms odiparcil’s eligibility to receive a Priority Review Voucher upon FDA approval of a new drug application (NDA) to be filed for odiparcil for the treatment of MPS VI.

Pierre Broqua, Ph.D., Chief Scientific Officer and cofounder of Inventiva, declared: “*This important designation from the FDA illustrates the severity of MPS VI in the pediatric population*. *We look forward to continued collaboration with the FDA in advancing odiparcil as a therapy in such a rare and life-threatening disease for which there is still a significant unmet medical need .*”

The FDA defines “rare pediatric diseases” as diseases with serious or life-threatening manifestations that primarily affect people who are less than 18 years old and that affect fewer than 200,000 people in the United States. Under the FDA's RPDD program, a sponsor who receives approval of an NDA or a biologics license application (BLA) for a product for the prevention or treatment of a designated rare pediatric disease may be eligible for a voucher, which can be redeemed to obtain priority review for a future submission of an NDA or BLA. Redeemed vouchers can reduce FDA review time from twelve to six months. Priority Review Vouchers may be used by the sponsor, or sold or transferred to a third party, with prices ranging from $67.5 million to $350 million since the first one was issued in 2009. The last voucher was sold in October 2018 for $80 million.[[1]](#footnote-1)

**About odiparcil**

Odiparcil is an orally-available small molecule that acts on the underlying cause of the symptoms of MPS. MPS is characterized by the accumulation of GAGs, which are important for the modulation of cell‑to‑cell signaling and the maintenance of tissue structure and function, in the lysosomes of cells. Due to genetic mutations, lysosomes in patients with MPS contain deficient versions of the enzymes necessary to break down GAGs. As a result, GAGs accumulate within the lysosomes, causing the latter to swell and interfere with the ordinary functioning of cells, thus leading to the symptoms associated with MPS. MPS is categorized by subtypes, depending on the enzyme that is deficient and the corresponding GAGs that accumulate. By modifying how GAGs are synthesized, odiparcil facilitates the production of soluble GAGs that can be excreted in the urine, rather than accumulating in cells. Specifically, odiparcil acts on chondroitin sulfate and dermatan sulfate, either or both of which accumulate in patients with MPS subtypes I, II, IVa, VI and VII.

Inventiva is currently evaluating odiparcil in a Phase IIa clinical study for the treatment of adult patients with MPS VI.

Odiparcil has been granted orphan drug status for the treatment of MPS VI by the FDA and the European Medicines Agency.

**About the Phase IIa iMProveS clinical trial**

The iMProveS (*Improve MPS treatment*) clinical trial is a 26-week Phase IIa clinical trial evaluating odiparcil for the treatment of adult patients with MPS VI. The primary endpoint of the trial is safety, as assessed by clinical and biological standard tests. Secondary endpoints include changes from baseline in leukocyte, skin and urinary GAG content, and improvements of activity and mobility, cardiovascular, lung and respiratory function, and vision and hearing impairments.

Inventiva expects to enrol 18 adult MPS VI patients currently receiving enzyme replacement therapy (ERT) in a double-blind placebo-controlled study and six patients not receiving ERT in an open label cohort at two sites in Europe. Results of the trial are expected in the second half of 2019.

**About Inventiva:** www.inventivapharma.com

Inventiva is a clinical-stage biopharmaceutical company focused on the development of oral small molecule therapies for the treatment of diseases with significant unmet medical needs in the areas of fibrosis, lysosomal storage disorders and oncology.

Leveraging its expertise and experience in the domain of compounds targeting nuclear receptors, transcription factors and epigenetic modulation, Inventiva is currently advancing two clinical candidates – lanifibranor and odiparcil – in non-alcoholic steatohepatitis (“NASH”) and mucopolysaccharidosis (“MPS”), respectively, as well as a deep pipeline of earlier stage programs.

Lanifibranor, its lead product candidate, is being developed for the treatment of patients with NASH, a common and progressive chronic liver disease. Inventiva is currently evaluating lanifibranor in a Phase IIb clinical trial for the treatment of this disease for which there are currently no approved therapies.

Inventiva is also developing odiparcil, a second clinical‑stage asset, for the treatment of patients with MPS, a group of rare genetic disorders. The Company is currently investigating odiparcil in a Phase IIa clinical trial for the treatment of adult patients with the MPS VI subtype.

In parallel, Inventiva is in the process of selecting an oncology development candidate for its Hippo signaling pathway program and is advancing pre‑clinical programs for the treatment of autoimmune diseases and idiopathic pulmonary fibrosis (“IPF”) in collaboration with AbbVie and Boehringer Ingelheim International respectively. AbbVie is investigating ABBV‑157, a clinical development candidate resulting from its collaboration with Inventiva, in a Phase I clinical trial for the treatment of moderate to severe psoriasis. Both collaborations entitle Inventiva to receive milestone payments upon the achievement of pre-clinical, clinical, regulatory and commercial milestones, in addition to royalties on any approved products resulting from the partnerships.

The Company has a scientific team of approximately 90 people with deep expertise in the fields of biology, medicinal and computational chemistry, pharmacokinetics and pharmacology. It also owns an extensive library of approximately 240,000 pharmacologically relevant molecules, 60% of which are proprietary, as well as a wholly‑owned research and development facility.

Inventiva is a public company listed on compartment C of the regulated market of Euronext Paris (Euronext: IVA – ISIN: FR0013233012). [www.inventivapharma.com](http://www.inventivapharma.com)

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*This press release contains forward-looking statements, forecasts and estimates with respect to the clinical development plans, business and regulatory strategy, and anticipated future performance of Inventiva and of the market in which it operates. Certain of these statements, forecasts and estimates can be recognized by the use of words such as, without limitation, “believes”, “anticipates”, “expects”, “intends”, “plans”, “seeks”, “estimates”, “may”, “will” and “continue” and similar expressions. Such statements are not historical facts but rather are statements of future expectations and other forward-looking statements that are based on management's beliefs. These statements reflect such views and assumptions prevailing as of the date of the statements and involve known and unknown risks and uncertainties that could cause future results, performance or future events to differ materially from those expressed or implied in such statements. Actual events are difficult to predict and may depend upon factors that are beyond Inventiva's control. There can be no guarantees with respect to pipeline product candidates that the candidates will receive the necessary regulatory approvals or that they will prove to be commercially successful. Therefore, actual results may turn out to be materially different from the anticipated future results, performance or achievements expressed or implied by such statements, forecasts and estimates.* *Given these uncertainties, no representations are made as to the accuracy or fairness of such forward-looking statements, forecasts and estimates. Furthermore, forward-looking statements, forecasts and estimates only speak as of the date of this press release.* *Readers are cautioned not to place undue reliance on any of these forward-looking statements.*

*Please refer to the “Document de référence" filed with the Autorité des Marchés Financiers on April 13, 2018 under   
n° R.18-013 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.*

1. Source: Biocentury May 4, 2018 “Voucher Equilibrium”; Biocentury November, 1st 2018 “Lilly buys Siga’s priority review voucher gained via smallpox approval”. [↑](#footnote-ref-1)