

## EMA's COMP grants Orphan Medicinal Product Designation to Lipopharma's Minerval® for the treatment of glioma.

*The European Medicines Agency's (EMA) Committee for Orphan Medicinal Products (COMP) has issued a positive opinion recommending orphan medicinal product (Orphan Drug, OD) designation for Minerval® for the treatment of glioma, a novel investigational compound designed by scientists at the University of the Balearic Islands (UIB) and developed by Lipopharma for its cancer indications.*

**Palma de Mallorca, October 21<sup>st</sup> 2011.** In its September meeting, EMA's COMP adopted a positive opinion recommending the designation as orphan medicinal products to the European Commission for Minerval® for the treatment of malignant brain tumours (glioma), the first product developed by Lipopharma on the basis of its innovative Membrane Lipid Therapy (MLT) technology platform developed by a team of scientists at the UIB led by Prof. Pablo V. Escriba.

**Orphan Drug (OD)** qualification in the European Union (EU) refers to pharmaceuticals that have been developed for the treatment of rare diseases that affects less than 5 patients in 10,000 habitants. Orphan Drug designation allows Lipopharma to follow a simplified clinical development for Minerval® and entitles the company to significant economic, scientific and regulatory benefits, including reduced fees and taxes, access to free protocol assistance and scientific advice by the EMA and a longer period of marketing exclusivity. **Vicenç Tur**, Chief Executive Officer and co-founder of Lipopharma said that *“the OD designation for our lead compound is a significant milestone for the development of a very promising new therapeutic treatment for a life threatening disease affecting some 40.000 persons in the EU every year, and that shows also a relatively high incidence in children up to 14 years old, being the second cause of cancer related death in this age group in Europe. It also constitutes a regulatory validation from the EMA for the innovative MLT platform we are using to design and develop next generation therapeutics for treating conditions such as cancer, neurodegeneration or inflammatory diseases, with very important medical needs unmet”*.

Lipopharma is the first Spanish company to obtain an OD designation from EMA for a new compound aimed at the treatment of malignant brain tumors (glioma). This process implied a thorough scientific evaluation including the establishment of **“sufficient justification”** to the EMA's COMP criteria, that the investigational compound *“...might be of significant benefit to those affected by the condition... in particular with regards to a potential clinically relevant advantage based on a new mechanism of action”*

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## ADDITIONAL INFORMATION

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### About Minerval®

**Minerval® (2-hydroxyoleic acid)** is a novel synthetic lipid that regulates the membrane lipid structure and composition of glioma cancer cells (including a marked increase in Sphingomyelin levels), inducing a translocation of **Ras** to the cytosol and subsequently inactivating the **MAPK** signalling pathway, which is found to be altered in almost 90% of adult glioblastomas. This deregulation of the MAPK pathway by Minerval® induces **differentiation** and **selective autophagy** in cancer cells but not in normal cells.

**Minerval®** is currently being tested for treatment of several types of cancer, most of them with very poor therapeutic prognosis, including lung cancer, malignant brain tumours, pancreatic cancer, leukemia, prostate cancer, colorectal cancer or breast cancer. Available preclinical results indicate that Minerval could become the first ever effective treatment for some of the most aggressive types of cancer such as of non-small cell lung cancer (NSCLC), glioma or pancreatic cancer. Minerval couples its powerful anticancer effect with absence of toxicity at therapeutics doses and its oral administration leads to a very attractive profile in cancer treatment. Moreover, as **Dr. Pablo V. Escribá**, inventor of Minerval®, Professor of Cell Biology at the UIB, co-founder and main scientific promoter of Lipopharma points out “*having a mechanism of action (MOA) totally different to most drugs currently used in oncology, brings a new hope in a very stringent research area, where advances have been very modest in the last decades for patients suffering from this deadly types of cancer. The novel MOA adds value as an agent that can be applied either as mono-therapy or in combination with other drugs*”. Lipopharma is preparing the first clinical trials in humans for the treatment of glioma to start in Q1 2012 and expects to have this compound in the market for treatment of glioma by 2014. Minerval® was designed by Dr. Escribá and his team of scientists at the Department of Biology of the University of the Balearic Islands and later licensed to Lipopharma for its oncology indications.

### About glioma

**Brain and other CNS tumours** represent about 1-2% of all newly diagnosed tumours in Europe and they account for about 2% of all cancer-related deaths. Brain tumours have a poor prognosis, and concomitantly, survival rate is amongst the lowest of all cancers. The 5-year and 10-year survival rates for all brain cancers are in the range of 10-20%, indicating the great need for efficacious therapies for this condition. Gliomas are the most common form of brain cancer in adults, representing about 60% of all primary brain tumours. Within gliomas, the most common is Glioblastoma Multiforme GBM, accounting for 40-45% of all diagnosed gliomas. Anaplastic Astrocytomas, Low-Grade Astrocytomas and oligodendrogliomas represent, respectively, 30-35%, 10% and 5-10% of all gliomas.

Current **Standard of Care** in Europe for the treatment of glioma includes surgical resection of the tumours (when possible) followed by Radiotherapy and concomitant chemotherapy, being temozolomide (TMZ) in almost all cases the chemotherapeutic agent used. In general terms, TMZ modestly improves the outcome of patients with glioma treated with radiotherapy/surgery as in patients treated with radiotherapy plus TMZ the median survival is about 14,5 months, compared with a median survival of 12 months for patients that only receive radiotherapy.

## **About MLT**

**Membrane-Lipid Therapy (MLT)** derives from a highly specialized scientific knowledge developed by Lipopharma's scientists and consists on the design of structure-based molecules that regulate the structure and functions of the membrane lipids, instead of targeting cellular proteins. This innovative know-how is Lipopharma's main expertise and lays on new discoveries made by Lipopharma's scientists related to the role of membrane lipids and membrane lipid structure on the regulation of localization and activity of membrane signalling proteins. This new therapeutic approach (MLT) constitutes a "disruptive innovation" which we believe will radically change the way diseases are fought, due to its exceptional combination of very high efficacy and lack of side effects.

## **About Lipopharma**

Lipopharma is an innovative biopharmaceutical company based in Palma de Mallorca (Spain), spun off from the University of the Balearic Islands, that specializes in the rational design and clinical development of pharmaceutical/nutraceutical products aimed at the prevention and treatment of human pathologies. The design of molecules is based on structure-function principles according to the unique MLT know-how developed by Lipopharma's scientific team. Lipopharma is also developing a portfolio of new products based on the MLT with potential applications in fields such as neurodegenerative diseases, inflammation or cancer. More detailed information on Lipopharma's pipeline can be found at the company's website ([www.lipopharma.com](http://www.lipopharma.com))

## **Disclaimer**

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