PRESS RELEASE

14th December, 2017

The European Medicines Agency (EMA) favorably comments on the orphan drug designation to a molecule with activity against Congenital Erythropoietic Porphyria, developed by CIC bioGUNE and ATLAS Molecular Pharma.

- Congenital erythropoietic porphyria is a rare, very serious and incurable disease.
- It is a repositioned drug, which has already been tested in humans for other specifications.
- Repositioning is obtained after years of basic research into the molecular mechanisms of the disease.
- This is the first designation of an orphan drug granted in the Basque Country.

The Committee for the designation of orphan drugs (COMP) of the European Medicines Agency (EMA) informs the **Basque company Atlas Molecular Pharma SL** about its **favorable opinion on the application of the drug L2.7.D7** for the treatment of **Congenital Erythropoietic Porphyria** (CEP). The EMA gives this recognition after a rigorous examination of the experimental activity data of the compound and constitutes an important advance in the development of a drug against this pathology, since it allows to initiate clinical trials with patients in the near future.

Orphan drugs are those destined for rare diseases that, traditionally, have not aroused the interest of the pharmaceutical industry for financial reasons, since they are intended for a small group of patients and, however, respond to public health needs .

The active principle developed is the result of many years of research at the **Center for Cooperative Research in Biosciences (CIC bioGUNE)** and is an example of how a **basic research project can naturally evolve towards a line of translational research**. In the laboratory of Protein Stability and Inherited Disease of the CIC bioGUNE, directed by Dr. Oscar Millet, they have dedicated eleven years to understand how some changes in the structure of certain proteins lead to their instability, which in the end instance triggers the

disease. In the words of Dr. Millet: "Understanding the molecular mechanism of the disease has made it possible to design a therapy based on **pharmacological chaperones**, which are molecules that bind to the defective protein, correcting its stability problem and reversing its pathogenic effects."

The biotechnology company ATLAS Molecular Pharma S. L., spin-off of the CIC bioGUNE, has developed an innovative platform for the screening of molecules for the discovery and validation of pharmacological chaperones (Chassys™). Within the *CEP project* of ATLAS, biochemical and biophysical experiments have been carried out with thousands of compounds, until a drug currently used in another pathology has also been demonstrated to show activity against CEP. Being a **repositioned drug**, the compound is approved by drug regulatory agencies for other specifications, **minimizing toxicity problems**. The repositioning success lies in the synergy between a highly qualified research team and the first level infrastructures existing in the CIC bioGUNE. It is worth noting the investments made in the animal house, which has hosted the experiments with animal models of PEC under the supervision of Dr. Joaquín Castilla and the Nuclear Magnetic Resonance Platform, which has allowed unequivocal information about the mechanism of action of the drug.

In the words of Dr. Emilio Díez, CEO of ATLAS: "The favorable report of the European Medicines Agency is a very important step for the development of L2.7.D7 for the treatment of Congenital Erythropoietic Porphyria. It ceritfies that this molecule has the appropriate pharmacological activity, both "in vitro" and "in vivo", having shown efficacy in the treatment of mice with a pathology similar to that of humans ".

Finally, it should be noted that this is the first designation of orphan drug granted in the Basque Country.

About the Congenital Erythropoietic Porphyria

Congenital erythropoietic porphyria (CEP) is a **rare disease** that affects less than one in a million people and is caused by a deficient activity of the enzyme uroporphyrinogen III synthase, which acts on the synthesis path of the heme group, which causes a reduction in the useful life of red blood cells (anemia) and an extreme photosensitivity of the skin, blistering and an increase in bacterial infections of the skin. Symptoms may appear during childhood or, in less severe cases, in adulthood. The existing treatments only alleviate the symptomatology without there being any type of effective curative therapy.

http://www.porfiria.org/

About ATLAS Molecular Pharma

Atlas Molecular Pharma, whose headquarters are located in the Scientific and Technological Park of Bizkaia, is dedicated to the development of **cutting-edge therapies for the treatment of rare diseases**, including CEP, prion diseases and type I tyrosianemia. The company has currently a staff of eight workers. Recently, Dr. Emilio Díez Monedero has joined ATLAS as CEO, with more than 25 years of international experience at GSK as Vice President of "Molecular Discovery Research" and Director of the Company's Basic Research Center in Spain.

http://www.atlasmolecularpharma.com/

About CIC bioGUNE

The Center for Cooperative Research in Biosciences CIC bioGUNE, based in the Scientific and Technological Park of Bizkaia, is a **biomedical research organization** that develops cutting-edge research at the interface between structural, molecular and cellular biology, with special attention to the study of the molecular basis of the disease, to be used in the development of new diagnostic methods and advanced therapies.

http://www.cicbiogune.es/