Atlas Molecular Pharma receives approval by the US Food & Drug Administration of an Orphan Drug for the treatment of Congenital Erythropoietic Porphyria

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DERIO, Spain.- Atlas Molecular Pharma, a private company founded in 2015 in Derio (Spain) as a spin-off from the Centre for Co-operative Research in Biosciences (CIC bioGUNE), announced today that the US Food & Drug Administration has granted the designation as Orphan-Drug of AMP-L2.7.D7 (ciclopirox) for *treatment of Congenital Erythropoietic Porphyria*. This recognition occurs only a few months after the European Medicine Agency and the European Commission approved ciclopirox as Orphan Medicinal Product for the treatment of this disease.

"The favourable report from the European Medicines Agency and the approval of Ciclopirox as an orphan drug by the European Commission and the FDA are very important steps in the development of Ciclopirox for the treatment of Congenital Erythropoietic Porphyria. ATLAS is currently securing appropriate funding for the clinical trials that demonstrate the benefit of ciclopirox in the treatment of this devastating disease. The new OMP and ODD status will facilitate the process and ultimately contribute to our goal of delivering Ciclopirox to patients suffering from Congenital Erythropoietic Porphyria at the earliest time possible", said Dr. Emilio Díez, CEO and CSO of Atlas Molecular Pharma.

Dr. Oscar Millet, Director of the Laboratory of Protein Stability and Inherited Disease of the CIC bioGUNE, said that "understanding the molecular mechanism of this disease has enabled us to design a therapy based on pharmacological chaperones, molecules that bind to the defective protein fixing its stability problem and reversing its pathogenic effects".

The EMA´s Orphan Medicinal Product program and the US FDA Orphan Drug designation provides orphan status to drugs and biologics that are being developed to address rare diseases or disorders that affect a very small percentage of the population. With the

recognition in Europe and in the US of Ciclopirox as an orphan drug, Atlas will qualify for various incentives that will facilitate the launching of clinical trials on patients in the near future. These incentives include scientific advice and high-quality clinical trial protocol assistance, leading to effective and acceptably safe medicines for the benefit of patients.

This is the first time that an orphan drug has been approved by the European Commission and the US Food & Drug Administration in the Basque Country.

About Congenital Erythropoietic Porphyria

Congenital erythropoietic porphyria (CEP) is a very rare inherited metabolic disorder resulting from the deficient function of the enzyme uroporphyrinogen III cosynthase (UROS), the fourth enzyme in the heme biosynthetic pathway. Due to the impaired function of this enzyme, excessive amounts of porphyrins accumulate, particularly in the bone marrow, plasma, red blood cells, urine, teeth, and bones. The major symptom of this disorder is hypersensitivity of the skin to sunlight and some types of artificial light, bone loss, and deformities. Available treatments only relieve the symptomatology and currently there is no curative therapy for this disease.

http://www.porphyriafoundation.com

About ATLAS Molecular Pharma

Atlas Molecular Pharma was founded in Derio (Spain) in September 2015 by the Centre for Co-operative Research in Biosciences (CIC bioGUNE) and the Venture Capital firm CRB Inverbio. Atlas Molecular Pharma is currently supported by an experienced group of life science investors, including Kereon Partners, Carlos Simón (Igenomix) and CRB Inverbio, in addition to CIC bioGUNE.

The Atlas business model is to discover first-in-class, innovative therapeutics for the treatment of Rare and Ultra-Rare Diseases and license them to larger partners who will deliver them to market for the patients that need them.

Atlas Molecular Pharma has a proprietary technological platform approach (CHASSYS™) that is being used as a “drug discovery engine” to deliver a class of therapeutics named “pharmacological chaperones” for the treatment of a range of Rare and ultra-Rare Diseases.

https://atlasmolecularpharma.com