



## **PRESS RELEASE**

### **ERYTECH Pharma's sickle cell treatment receives positive opinion for Orphan Drug Designation from the European Medicines Agency**

**Lyon (France) - June 13, 2012 - ERYTECH Pharma, a late stage biopharmaceuticals company focused on orphan oncology and rare diseases, announces that the Committee for Orphan Medicinal Products (COMP) of the European Medicines Agency (EMA) adopted a positive opinion to grant Orphan Drug Designation (ODD) to its investigational product ENHOXY<sup>®</sup> for sickle cell disease.**

ENHOXY<sup>®</sup>, human erythrocytes encapsulating inositol hexaphosphate, enhances the oxygenation properties of red blood cells by reducing their oxygen-hemoglobin affinity and allowing them to release more oxygen. Hypoxia, lack of oxygen in the blood, is associated with the initiation of cell sickling and severe cell sickling crisis. Total blood exchanges, using several blood units, are often needed to reverse hypoxic conditions and prevent crises in patients with this disease. ENHOXY<sup>®</sup> targets to reduce the number of blood transfusions needed.

An ODD provides ten years of market exclusivity after marketing authorization in all member states of the European Union, a streamlined regulatory review process and important fee reductions.

"I am delighted with this positive opinion. This ODD is an important step for the development of the ENHOXY<sup>®</sup> product that, we believe, can bring significant benefit to the patients suffering from this debilitating disease. With a strong preclinical package, we are now scaling up manufacturing under cGMP to be ready to initiate clinical trials next year" said Dr Yann Godfrin, Executive Vice President and Chief Scientific Officer.

"ENHOXY<sup>®</sup> is a good example of the potential of our RBC based platform to bring meaningful benefit to patients with unmet needs in oncology and rare diseases." adds Pierre-Olivier Goineau, Chief Executive Officer, "While we are focused on advancing the oncology franchise, indications like sickle cell disease present

significant opportunity for value creation to ERYTECH, value we intend to capture through partnerships and alliances with therapy specialists”.

### **About ERYTECH**

ERYTECH Pharma SA is a late-stage French biopharmaceutical company developing medicinal products for orphan oncology and rare diseases. The company's proprietary core technology is based on the use of human red blood cells (RBCs) to improve the pharmacokinetic (PK) and pharmacodynamic (PD) properties of therapeutic molecules.

ERYTECH's lead product, GRASPA<sup>®</sup> for treatment of Acute Lymphoblastic Leukemia (ALL), holds orphan designation in Europe and the US, and is in pivotal Phase II/III clinical trial in Europe. Initial sales from early access programs are expected in 2012 and the first regional distribution and marketing deal has been signed with Teva Pharmaceuticals. ERYTECH has established a cGMP approved manufacturing facility that can meet the demand for GRASPA<sup>®</sup> products in Europe.

The company is expanding the use of its technology in oncology to Acute Myeloid Leukemia and solid tumors, and outside oncology in enzyme replacement therapy, immunology and haematology indications.

### **About Sickle Cell Anemia (SCA)**

SCD is a genetic disease causing amino acid substitution on the beta-chain of haemoglobin. This pathology is characterized by abnormal blood rheology and periods of painful vascular occlusive crises.

This disorder is heterogeneous, with clinical manifestations including chronic haemolysis, an increased susceptibility to infections and vaso-occlusive complications often requiring medical care. Patients with SCD can develop specific and sometimes life-threatening complications, as well as extensive organ damage reducing both their quality of life and their life expectancy. About 80.000 patients suffer of sickle cell anemia in Europe and about 100.000 in USA.

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