

## **ERYTECH announces second positive DSMB review of its Phase IIb study in Acute Myeloid Leukemia**

- Independent experts have analyzed the safety data of the first 60 patients treated
- As in the first analysis, on the first 30 patients, the experts unanimously recommended continuation of the trial without modification

---

**Lyon (France), August 27, 2014** – ERYTECH Pharma (Euronext Paris: FR0011471135 - ERYP), the French biopharmaceutical company that develops innovative ‘tumor starvation’ treatments for acute leukemia and other oncology indications with unmet medical needs, announces that an independent Data and Safety Monitoring Board (DSMB) completed its second safety assessment of the company’s Phase IIb study in Acute Myeloid Leukemia (AML) and unanimously recommended continuation of the trial without modification.

The GRASPA-ML study is a multicentre, randomized, controlled Phase IIb trial evaluating the efficacy and tolerability of GRASPA® in the treatment of newly diagnosed AML patients over 65 years old that are unfit for intensive chemotherapy. In this 123 patient study, one-third of the patients receive the current standard treatment (low-dose cytarabine) and two-thirds receive low-dose cytarabine plus GRASPA®. The study was initiated in March 2013 and is performed in collaboration with Orphan Europe (Recordati Group), ERYTECH’s partner for the commercialization of GRASPA® in 38 European countries.

A DSMB is an external committee of independent clinical research experts who review data in ongoing clinical trials with particular attention to safety. This second DSMB assessment was based on a pre-planned safety analysis on the first 60 patients included in the study and with a minimum of 1 month follow-up. A first DSMB assessment took place at the end of 2013 when 30 patients had been treated in the study. The next step will be another DSMB analysis, this time for safety and futility, when 60 patients will have experienced an event in the study. This analysis is anticipated towards the end of this year.

*“We are pleased to see that this second DSMB review confirmed the safety profile of the product. This is all the more important as this is the first time that a large group of elderly AML patients is receiving repeated doses of an asparaginase-based product in a controlled clinical trial. The use of asparaginase has to date been very limited in this indication due to the toxicity of the current forms of asparaginase. Thanks to the encapsulation in the red blood cells we are enabling the use of asparaginase in the treatment of these very fragile patients”,* comments Yann Godfrin, co-founder and CSO of ERYTECH Pharma.

**About ERYTECH and ERY-ASP/GRASPA®:** [www.erytech.com](http://www.erytech.com)

Created in Lyon in 2004, ERYTECH is a French biopharmaceutical company providing new prospects for cancer patients, particularly those with acute leukemia and selected solid tumors.

By encapsulating the asparaginase enzyme in red blood cells, ERYTECH has developed ERY-ASP/GRASPA®, an original treatment that targets cancer cells through “tumor starvation” while significantly reducing the side effects for patients. ERY-ASP/GRASPA® is currently completing Phase III clinical development in Acute Lymphoblastic Leukemia (ALL) and is in Phase IIb clinical trial in Acute Myeloid Leukemia (AML) in Europe. The product is also in Phase I/II clinical development in ALL in the USA.

Every year about 50,000 patients are diagnosed with Acute Lymphoblastic Leukemia (ALL) or Acute Myeloid Leukemia (AML), the two forms of acute leukemia. Today, for about 80% of these patients, mainly adults and relapsing patients, current forms of asparaginase cannot be used due to their toxicity. With a presumed improved safety profile, ERY-ASP is being developed to allow all leukemia patients to be treated, even the most fragile ones, representing a market opportunity of more than EUR 1 billion..

The company is also developing other indications in solid tumors and certain orphan indications outside oncology. The company is currently launching a Phase II study in pancreas cancer and it exploring other solid tumor indications.

ERYTECH has obtained orphan drug designations for ERY-ASP/GRASPA® in ALL, AML and pancreas cancer, both in Europe and the USA, and has its own GMP-approved and operational manufacturing site in Lyon (France), and a site for clinical production in Philadelphia (USA).

The company has concluded licensing and distribution partnership agreements for ALL and AML in Europe with Orphan Europe (Recordati Group), and for ALL with TEVA in Israël.

*ERYTECH is listed on Euronext regulated market in Paris. (ISIN code: FR0011471135, ticker: ERYP) and is part of the CAC Healthcare, CAC Pharm. & Bio and Next Biotech indexes.*

### **About Acute Myeloid Leukemia (AML)**

Acute Myeloid Leukemia (AML) is an aggressive form of leukemia (blood or bone marrow cancer) that is characterized by a rapid and abnormal proliferation of myeloid precursor cells. AML usually progresses quickly and, if not treated, can be fatal within a few months. With about 34 000 new patients per year in Europe and the USA, AML is the most common type of acute leukemia. Affecting mainly the adult and senior patient population that often cannot tolerate the existing forms of asparaginase products, AML represents one of the highest mortality rates among all type of cancers and an important unmet medical need. The median age of patients affected by AML is 67 years.

### **Forward-looking information**

This document may contain forward-looking statements and estimates with respect to the financial situation, the results of operations, the strategy, the project and to the anticipated future performance of ERYTECH 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. They include all matters that are not historical facts. Such statements, forecasts and estimates are based on various assumptions and assessments of known and unknown risks, uncertainties and other factors, which were deemed reasonable when made but may or may not prove to be correct. Actual events are difficult to predict and may depend upon factors that are beyond the Company's control. Therefore, actual results, the financial condition, performance or achievements of ERYTECH, or industry results, may turn out to be materially different from any future results, performance or achievements expressed or implied by such statements, forecasts and estimates. Documents filed by ERYTECH Pharma with the French Autorité des Marchés Financiers ([www.amf-france.org](http://www.amf-france.org)), also available on our website ([www.erytech.com](http://www.erytech.com)) describe such risks and uncertainties. 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 the publication of this document. ERYTECH disclaims any obligation to update any such forward-looking statement, forecast or estimates to reflect any change in the Company's expectations with regard thereto, or any change in events, conditions or circumstances on which any such statement, forecast or estimate is based, except to the extent required by French law.

### **CONTACTS**

#### **ERYTECH**

**Gil Beyen**  
*Chairman & CEO*  
**Pierre-Olivier Goineau**  
*Vice President & COO*  
Tel: +33 4 78 74 44 38  
[investors@erytech.com](mailto:investors@erytech.com)

#### **NewCap**

**Julien Perez / Emmanuel Huynh**  
Investor and press relations  
Tel: +33 1 44 71 98 52  
[erytech@newcap.fr](mailto:erytech@newcap.fr)

