

## **ERYTECH completes enrollment in its Phase III study in Acute Lymphoblastic Leukemia**

- **Randomized controlled pivotal study with 80 patients treated**
- **58 investigator sites in various European countries (France, Belgium, Spain)**
- **Results to be available in Q3 2014**
- **Last step before launch of the registration of GRASPA® in Europe**

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**Lyon (France), September 10, 2013** – ERYTECH (NYSE Euronext Paris: FR0011471135 - ERYP), a French biopharmaceutical company that develops innovative treatments for acute leukemia and other oncology indications with unmet medical needs, announces the completion of the enrollment of patients in its pivotal Phase III study in Acute Lymphoblastic Leukemia (ALL).

The study compares ERYTECH's lead product, GRASPA®, to native asparaginase in a randomized controlled and multicenter clinical trial on 80 children and adults suffering from relapsing or refractory ALL.

Involving 58 investigator sites in France, Belgium and Spain, this study is the last step before the launch of the registration process with a view to obtaining central marketing authorization in Europe.

As a reminder, the study was launched in 2009 as a Phase II/III study with an adaptive design protocol. During the first quarter of this year an independent Data Safety Monitoring Board (DSMB) reviewed the data of the first 60 patients and recommended the transition to Phase III and the continuation of the study without changes to the protocol. Full results of this pivotal study are expected in Q3 of 2014.

Asparaginase is a mainstay in the treatment of children suffering from ALL, but its use is limited in older and fragile patients due to its toxicity profile. GRASPA® is a new formulation of asparaginase, with a safer and broader range of clinical use than existing forms due to the entrapment and protection of the enzyme inside red blood cells. GRASPA® is intended to satisfy the unmet medical needs of older and frail patients suffering relapses and others for whom the other forms of asparaginase represent a risk.

GRASPA® has already successfully completed a Phase I/II study in relapsing children and adults and a Phase II study in newly diagnosed ALL patients over 55 years old. Both studies have demonstrated an improved safety profile and promising efficacy data.

Earlier this year, the product also received FDA clearance to start clinical development in ALL in the US. The company expects to begin the enrollment of patients in the Phase Ib study in the coming months.

For the indication in ALL, GRASPA® holds orphan drug designation in Europe and the USA.

*"We have highly appreciated the motivation and interest of the investigating clinicians for this pivotal study. This has not only allowed to stay within the planned timelines, but also to confirm the substantial medical need. We would like to take this opportunity to thank all people involved in this landmark study, especially the clinicians and the patients",* said Dr Yann Godfrin, co-founder and Chief Scientific Officer of ERYTECH.

*"This achievement represents a major milestone for us and our partner Orphan Europe – Recordati; few products reach this stage in oncology. We should be able to launch the registration process in Europe in the fall of this year as planned",* adds Gil Beyen, Chairman and CEO of ERYTECH.

## About Acute Lymphoblastic Leukemia

Acute Lymphoblastic Leukemia (ALL) is an aggressive form of leukemia (blood or bone marrow cancer) that is characterized by a rapid and abnormal proliferation of lymphoid precursor cells. ALL usually progresses quickly and, if not treated, can be fatal within a few months. Every year about 10,000 people are diagnosed with ALL in Europe (EU27) and about 6,000 in the US. Thanks to the development of new therapies and medicines, notably asparaginase, the prognosis for children affected by ALL has increased considerably with 5 year survival rates around 90%. For older patients (adults and seniors) and patients in relapse, who often don't tolerate existing therapies, overall long-term survival remains among the lowest in the field of cancer (10% to 30%), leaving an important unmet medical need.

## About ERYTECH: [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. 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, there is no adequate solution due to the toxicity of existing treatments, representing a market opportunity of more than EUR 1 billion. By encapsulating the asparaginase enzyme in red blood cells, ERYTECH has developed GRASPA®, an original and effective treatment that targets leukemia cells through "starvation" while significantly reducing the side effects for patients, and allowing all patients to be treated, even the most fragile ones. 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). ERYTECH has concluded distribution partnership agreements for Europe with Orphan Europe (Recordati Group), and with TEVA for Israel. In the United States, ERYTECH is launching a Phase Ib clinical trial in ALL, after having received approval from the US FDA. The company is also developing other indications in solid tumors and certain orphan indications outside oncology. ERYTECH has its own GMP-approved and operational manufacturing site.

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

## Forward-looking information

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