

ERYTECH positions its lead product in solid tumors and launches a Phase II clinical study in pancreatic cancer

- **Mode of action of ERYASP¹ confirmed in different solid tumor indications**
- **Clinical trial application submitted for Phase II study in pancreatic cancer following EMA Scientific Advice**
- **Potential to target major unmet medical needs representing a multi-billion market opportunity**

Lyon (France), January 7th, 2014 – ERYTECH Pharma (Euronext Paris: FRO011471135 - ERYP), a French biopharmaceutical company that develops innovative treatments for acute leukemia and other oncology indications with unmet medical needs, provides an update on its development plans to broaden the scope of its lead product ERYASP into the large field of solid tumors and announces the launch of a Phase II study in pancreatic cancer.

After having performed multiple clinical studies with promising results in acute leukemia, the research and development teams of ERYTECH have, in collaboration with researchers at the world renowned MD Anderson Cancer Center in Houston, worked intensively at confirming the mode of action and the potential of ERYASP in other cancer indications. In extensive testing on a large number of tumor samples, it was found and published in a high ranking peer reviewed journal² that 50% to 90% of known solid tumors are eligible for treatment through the concept of tumor starvation by administration of asparaginase. ERYTECH has demonstrated that solid tumors such as pancreatic, liver, bladder and ovarian cancer are potentially sensitive to an asparaginase-based treatment, which represents a multi-billion market opportunity³ for the product, potentially an order of magnitude much larger than the already sizeable opportunity in acute leukemia.

The company has retained pancreatic cancer, a very aggressive form of cancer with few treatment options, as the lead indication for ERYASP in solid tumors. In Europe and the USA alone, every year about 140,000 patients are newly diagnosed with pancreas cancer. With an overall 5 year survival of 6 to 10%, pancreas cancer is one of the most aggressive forms of cancer.

Having already successfully completed a Phase I study in late stage pancreas cancer, in which the tolerability of ERYASP has been confirmed in this very fragile patient population, ERYTECH has decided to continue the development in solid tumors by performing a Phase II study in second line pancreas cancer. In a study of about 100 patients, ERYASP in addition to the best standard of care will be compared to the best standard of care alone in a 2-to 1 randomization.

Thanks to an exclusive license from the National Institutes of Health (NIH) in the USA, ERYTECH has a proprietary companion test allowing to identify the potential responders to treatment with asparaginase, in view of offering personalized treatment.

¹ ERYASP is the codename for ERYTECH's lead product, "Asparaginase encapsulated in red blood cells". The product is named GRASPA[®] for indication in acute leukemia in Europe

² Dufour E et al., *Pancreas*, 2013

³ Company data

Scientific advice has been obtained from the European Medicines Agency (EMA) and a clinical trial application (CTA) has subsequently been submitted to the ANSM, the French authority for drug safety. Professor Pascal Hammel, gastro-enterologist and oncologist at Hôpital Beaujon (Clichy-Paris, France), will be the principal investigator of the study. Patient enrollment is expected to start in Q2 2014. ERYASP already holds orphan drug designation for the treatment of pancreas cancer in Europe and the USA.

“The extensive research work performed by our teams in collaboration with leading scientists at the MD Anderson Cancer center confirms the interest of asparaginase based products in the treatment of solid tumors. Our unique formulation in red blood cells opens the perspective to offer a therapeutic alternative to a large number of these cancer patients.” said Dr Yann Godfrin, CSO at ERYTECH.

“We look forward to evaluating the efficacy of the ERYASP product in patients affected by non-resectable pancreas cancer. Asparagine tumor starvation is an interesting approach to treat cancer and can contribute to enlarge our therapeutic arsenal against this terrible disease. In addition the Phase I clinical data showed a good safety profile so far.” said Professor Pascal Hammel, gastro-enterologist and oncologist at Hôpital Beaujon.

About pancreas cancer:

Pancreatic cancer is a disease in which malignant (cancer) cells are found in the tissues of the pancreas. Every year about 45,000 patients are diagnosed with pancreatic cancer in the US, and about 95,000 in Europe. According to the American Cancer Society, for all stages of pancreatic cancer combined, the one-year relative survival rate is 20%, and the five-year rate is 6% to 10%. Pancreatic cancer is currently the fourth most common cause of cancer death in the EU for men and women. Death rates from the disease are predicted to rise from 7.85 in 2009 to 8.01 in 2013 per 100,000 among men, and from 5.33 to 5.54 per 100,000 among women in same period. In fact, the pancreas is the only major cancer site for which no improvements in mortality rates is predicated⁴.

About ERYTECH and ERYASP/GRASPA®: 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.

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. By encapsulating the asparaginase enzyme in red blood cells, ERYTECH has developed ERYASP/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, representing a market opportunity of more than EUR 1 billion. ERYASP/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). The product also received FDA clearance to start clinical development in ALL in the USA. ERYTECH has concluded distribution partnership agreements for Europe with Orphan Europe (Recordati Group), and with TEVA for Israel.

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 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

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,

⁴ Malvezzi M et al., *Annals of Oncology* 2013, 1-9

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), also available on our website (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 and CEO

Pierre-Olivier Goineau

Vice President and COO

Tel: +33 4 78 74 44 38

investors@erytech.com

NewCap.

Julien Perez / Emmanuel Huynh

Investor and press relations

Tel: +33 1 44 71 98 52

erytech@newcap.fr

