

ERYTECH receives Orphan Drug Designation by the FDA for ERY-ASP in Acute Myeloid Leukemia

- Seventh Orphan Drug Designation (ODD) for ERYTECH
- All three of ERYTECH's lead indications now benefit from ODD in Europe and the USA

Lyon (France), April 2nd, 2014 – ERYTECH Pharma (Euronext Paris: FR0011471135 - ERYP), a French biopharmaceutical company that develops innovative treatments for acute leukemia and other oncology indications with unmet medical needs, announces that its lead product GRASPA[®]/ERY-ASP¹ has been granted Orphan Drug Designation by the U.S. Food and Drug Administration (FDA) for the treatment of Acute Myeloid Leukemia (AML), an aggressive form of blood cancer.

Orphan Drug Designation (ODD) is generally granted to drugs or biologics intended for treatment of rare diseases and disorders of high unmet medical need, affecting fewer than 200,000 people in the USA. This designation conveys special incentives to the sponsor, including seven years of US market exclusivity for the drug or biologic upon FDA approval, a prescription drug user fee waiver, and certain tax credits.

AML, the most common form of acute leukemia, affects about 34,000 new patients per year in the US and Europe, mainly adults and older patients, for which few therapeutic options are available today.

ERY-ASP, in Europe known as GRASPA[®], is a new enzyme formulation of L-asparaginase, with a safer and broader range of clinical use than existing forms due to the entrapment and protection of the enzyme inside homologous red blood cells. ERY-ASP is in Phase III clinical trial for Acute Lymphoblastic Leukemia (ALL) and in Phase IIb trial for AML in Europe. A Phase I study in adult ALL is being launched in the USA as well as a Phase II study in pancreas cancer in Europe.

The rationale for using L-asparaginase in AML is strong. However, as the median age of AML patients is about 70 years old, a large majority of them are too fragile to tolerate the current asparaginase formulations. Thanks to its better safety profile, ERY-ASP aims to make asparaginase available also to AML patients.

The new Orphan Drug Designation is the seventh ODD for ERYTECH. The dossier was filed after the positive safety review by an independent Data Safety Monitoring Board (DSMB) of the first 30 patients treated in the ongoing Phase IIb study.

ERY-ASP now benefits from ODD in all three of its lead indications, ALL, AML and pancreas cancer, both in Europe and the USA.

"We are very pleased by the granting of this additional Orphan Drug Designation" said Dr Yann Godfrin, co-founder and Chief Scientific Officer of ERYTECH. "We have been encouraged by the results from our work in Acute Lymphoblastic Leukemia, and are committed to further clinical evaluation of the product in other indications of high unmet need. Acute Myeloid Leukemia and solid-tumor cancers are investigated to develop a safe and effective therapy, providing a superior treatment outcome. Orphan Drug Status provides certain benefits that can support us in achieving this goal."

¹ GRASPA[®] is the future tradename of the product in Europe; ERY-ASP is the development name in other regions

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.

About ERYTECH and ERY-ASP/GRASPA®: www.erytech.com

Founded 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 ERY-ASP/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. 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 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, 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.

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