

## ERYTECH Reports Determination of the Recommended Pivotal Phase 3 Dose of eryaspase in its U.S. Phase 1 Study in First Line Adult ALL

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- **Dose of 100 U/kg, which is the same dose used in other studies with eryaspase in adult acute lymphoblastic leukemia (ALL), acute myeloid leukemia (AML) and metastatic pancreatic cancer patients, was recommended for further evaluation of eryaspase in pivotal study in first line adult ALL**

LYON, France--(BUSINESS WIRE)--Sep. 28, 2017-- Regulatory News:

**ERYTECH Pharma(Paris:ERYP) (ADR:EYRY), a clinical-stage biopharmaceutical company developing innovative therapies by encapsulating therapeutic drug substances inside red blood cells, today announced the determination of the recommended pivotal Phase 3 dosing from its U.S. Phase 1 dose escalation study with eryaspase (GRASPA®) in first line treatment of adult ALL patients.**

The U.S. Phase 1 study with eryaspase (GRASPA) is an open label dose escalation study evaluating the safety of eryaspase in combination with CALGB 8811 protocol for first line treatment of adult ALL patients. The study is performed at five clinical sites across the United States. Prof. Dr. Richard Larson, director of the Hematologic Malignancies Clinical Research Program at the University of Chicago, is the principal investigator of the study.

ERYTECH recently announced that all patients had been treated in the third dose escalation cohort of this Phase 1 study. The steering committee of the study reviewed the safety data of all three treatment cohorts and agreed to pursue further development at the dose level of 100 U/kg. This dose level had been previously recommended following ERYTECH's Phase 2 study in elderly ALL patients. It is also the dose level used in the Phase 2b study in second line, metastatic pancreatic cancer, that recently reported positive efficacy and safety results, and in the ongoing Phase 2b study in AML, from which top-line results are expected by the end of this year.

In parallel with running an expansion cohort of this Phase 1 study at this recommended dose, ERYTECH will potentially initiate the steps toward the launch of a pivotal Phase 3 study in first line adult ALL patients at this dose level.

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

Founded in Lyon, France in 2004, ERYTECH is a clinical-stage biopharmaceutical company developing innovative therapies for rare forms of cancer and orphan diseases. Leveraging its proprietary ERYCAPS platform, which uses a novel technology to encapsulate therapeutic drug substances inside red blood cells, ERYTECH has developed a pipeline of product candidates targeting markets with high unmet medical needs. ERYTECH's initial focus is on the development of products that target the amino acid metabolism of cancer, depriving them of nutrients necessary for their survival.

The company's lead product, eryaspase, also known under the trade name GRASPA®, consists of an enzyme, L-asparaginase, encapsulated inside donor-derived red blood cells. L-asparaginase depletes asparagine, a naturally occurring amino acid essential for the survival and proliferation of cancer cells. L-asparaginase has been a standard component of multi-agent chemotherapy for the treatment of acute lymphoblastic leukemia (ALL), but side effects limit treatment, especially in adults and patients with weak performance status. With its improved safety profile, eryaspase aims to provide L-asparaginase to patients who cannot tolerate current non-encapsulated asparaginases.

Eryaspase achieved positive efficacy and safety results in a Phase 2 study in elderly patients with ALL, and a Phase 2/3 study in children and adults with relapsed or refractory ALL. ERYTECH believes that the positive results of its Phase 2b clinical study in second-line metastatic pancreatic cancer are significant indicators of eryaspase as a potential treatment approach in solid tumors. ERYTECH also has an ongoing Phase 1 clinical study of eryaspase in the United States in adults with newly diagnosed ALL and a Phase 2b clinical study in Europe in elderly patients with newly diagnosed acute myeloid leukemia (AML), each in combination with chemotherapy.

ERYTECH produces eryaspase at its own GMP-approved and operational manufacturing site in Lyon (France), and at a site for clinical production in Philadelphia (USA). ERYTECH has entered into licensing and distribution partnership agreements for eryaspase for ALL and AML in Europe with Orphan Europe (Recordati Group), and for ALL in Israel with TEVA, which will market the product under the GRASPA® brand name. The European Medicines Agency (EMA) and the U.S. Food and Drug Administration (FDA) have granted orphan drug designations for eryaspase for the treatment of ALL, AML and pancreatic cancer.

In addition to eryaspase, ERYTECH is developing two other product candidates, erymethionase and eryminase, that focus on using encapsulated enzymes to target cancer metabolism and induce tumor starvation. ERYTECH is also exploring the use of its ERYCAPS platform for developing cancer immunotherapies (ERYMMUNE) and enzyme replacement therapies (ERYZYME).

*ERYTECH is listed on Euronext regulated market in Paris (ISIN code: FR0011471135, ticker: ERYP) and is part of the CAC Healthcare, CAC Pharma & Bio, CAC Mid & Small, CAC All Tradable, EnterNext PEA-PME 150 and Next Biotech indexes. ERYTECH is also listed in the U.S. under an ADR level 1 program (OTC, ticker EYRY).*

### Forward-looking information

This press release contains forward-looking statements, forecasts and estimates with respect to the clinical development plans, business and regulatory strategy, and 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

ERYTECH's control. There can be no guarantees with respect to pipeline product candidates that the candidates will receive the necessary regulatory approvals or that they will prove to be commercially successful. Therefore, actual results may turn out to be materially different from the anticipated 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 ERYTECH's 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 this press release. Readers are cautioned not to place undue reliance on any of these forward-looking statements. ERYTECH disclaims any obligation to update any such forward-looking statement, forecast or estimates to reflect any change in ERYTECH'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 law.

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