

## **ERYTECH Announces Positive Full Results from Phase 2b Study of Eryaspase in Combination with Chemotherapy for Treatment of Metastatic Pancreatic Cancer in Second-Line**

- Full data demonstrates statistically significant improvement in both overall survival and progression-free survival for eryaspase as second-line treatment of metastatic pancreatic cancer
- Data to be presented at the European Society for Medical Oncology (ESMO) Annual Meeting in Madrid (September 8-12)
- Meeting with U.S. FDA upcoming to discuss the design of Phase 3 study
- Company to host Investor & Analyst Event with live webcast, Monday, September 11, 6:15 p.m. CEST/12:15 p.m. EDT

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**Lyon (France), September 8, 2017** – ERYTECH Pharma (Euronext Paris - ERYP), a clinical-stage biopharmaceutical company developing innovative therapies by encapsulating therapeutic drug substances inside red blood cells, today announced the presentation of the full data from its Phase 2b study evaluating eryaspase (GRASPA®) in combination with chemotherapy for the treatment of metastatic pancreatic cancer. The open-label, multi-center, randomized Phase 2b clinical study met its co-primary endpoints and demonstrated significant improvement in both overall survival (OS) and progression-free survival (PFS). The results will be presented during the [European Society for Medical Oncology \(ESMO\) Annual Meeting in Madrid](#).

The Phase 2b study evaluated eryaspase, L-asparaginase encapsulated in red blood cells, as a second-line treatment in combination with chemotherapy in 141 patients suffering from metastatic pancreatic cancer. In this study, conducted in France, eryaspase was added to the current standard of care (gemcitabine or FOLFOX) and compared to the standard of care alone in a 2-to-1 randomization. Approximately 90% of patients received gemcitabine. Baseline characteristics and patient demographics were similar between the two treatment groups.

As reported in topline results earlier this year, the study met its co-primary endpoints of OS and PFS with Hazard Ratios (HR) below 0.85 in patients with no or low asparagine synthetase expression (ASNS 0/1), approximately 70% of the study population, and demonstrated statistically significant improvements of OS and PFS in the entire patient population. The associated sensitivity analyses and subgroup evaluations indicate the consistent treatment benefit with eryaspase across the treated populations.

Principal Investigator Professor Pascal Hammel, gastroenterologist-oncologist and head of the Oncology Unit at Beaujon Hospital in Paris, commented, *“The full results of this study are highly encouraging and support eryaspase as a potential treatment option for patients with metastatic pancreatic cancer in the second-line setting.”*

Highlights of the study results include:

- Co-primary endpoints met:
  - HR of 0.65 for OS and 0.72 for PFS in the ASNS 0/1 patient population
- Statistically significant improvement of OS and PFS in the entire patient population:
  - HR of 0.60 for OS (95% CI; 0.40, 0.88) (p=0.009)

- median OS of 26.1 weeks (95% CI; 21.0, 28.4) in the eryaspase arm vs. 19.0 weeks (95% CI; 12.3, 26.3) in the standard of care arm
    - one-year survival of 14.8% vs. 3.0%, respectively
  - HR of 0.59 for PFS (95% CI; 0.40, 0.89) (p=0.011)
    - median PFS of 8.6 weeks (95% CI; 7.6, 14.6) in the eryaspase arm vs. 7.0 weeks (95% CI; 6.1, 7.6) in the standard of care arm
    - 16.9% of patients without disease progression at 24 weeks vs. 5.8%, respectively
- Improved objective response rate (ORR) and disease control rate (DCR) in the entire patient population:
  - ORR of 11.6% in the eryaspase arm vs. 6.5% in the standard of care arm
  - DCR of 47.4% in the eryaspase arm vs. 23.9% in the standard of care arm
- Patients with high ASNS-expressing tumors (ASNS2/3) had a worse prognosis, but also a better relative treatment benefit:
  - HR of 0.45 for OS and 0.38 for PFS
  - DCR of 51.7% in the eryaspase arm vs. 7.1% in the standard of care arm
- The toxicity profile was similar in the two treatment arms:
  - The percentage of patients with at least one Grade 3 or 4 adverse event (AE) was 77% in the eryaspase-treated arm compared to 86% in the control arm. The most common Grade 3 or 4 AEs were: increased gamma-glutamyl transferase (17% vs. 25%), neutropenia (13% vs. 11%), general health deterioration (13% vs. 2%), and thrombocytopenia (10% vs. 7%), respectively.
  - The percentage of patients with at least one serious adverse events (SAE) was 45% in the eryaspase-treated arm compared to 50% in the control arm. The most common SAEs were: general health deterioration (9% each), gastro-intestinal hemorrhage (2% vs. 7%, respectively).

Dr. Iman El-Hariry, Chief Medical Officer of ERYTECH, stated, *“Despite intense research efforts, limited progress has been made toward increased overall survival and metastatic pancreatic cancer remains a high unmet medical need. We are quite impressed with this study outcome, particularly with the overall survival advantage demonstrated in the eryaspase arm. These results underscore the importance of targeting the metabolic pathways in pancreatic cancer and potentially other solid tumors.”*

*“We are very pleased by the results from this landmark study. The full picture emerging from these data shows a robust clinical benefit in this particularly difficult-to-treat and highly morbid form of cancer,”* said Gil Beyen, Chairman and CEO of ERYTECH. *“Eryaspase adds an entirely new mode of action to the fight against this terrible disease and opens avenues to other solid tumor indications. We are working with the regulatory agencies to develop a Phase 3 plan in pancreatic cancer, and we are exploring other solid tumor indications for our product candidate.”*

The full poster presentation will be accessible on September 8, 2017 within the “Investors” section of ERYTECH’s website at [www.investors.erytech.com](http://www.investors.erytech.com).

ERYTECH will also host an investor and analyst event on Monday, September 11 at 6:15 p.m. CEST at ESMO 2017, in Madrid. Pre-registration for the live event at ESMO 2017 is required. To RSVP, please contact Janhavi Mohite at [jmohite@theruthgroup.com](mailto:jmohite@theruthgroup.com).

For those unable to attend, a live webcast will be accessible at the start of the event starting at **06:30pm CEST**, and is available for replay on the “Investors” of the ERYTECH’s website at <http://erytech.com/webcast.html>.

## About pancreatic cancer:

Pancreatic cancer is a disease in which malignant (cancer) cells are found in the tissues of the pancreas. Every year, there are approximately 150,000 new cases of pancreatic cancer diagnosed in Europe and the United States. Pancreatic cancer is a particularly aggressive cancer, with a five-year survival rate of approximately 9%. It is currently the fourth leading cause of cancer death in the United States and is projected to rise to the second leading cause by 2030. Limited therapeutic options are currently available for this indication, thereby reinforcing the need to develop new therapeutic strategies and rational drug combinations with the aim of improving overall patient outcomes and quality of life.

## 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 degrades asparagine, a naturally occurring amino acid essential for the survival and proliferation of cancer cells. L-asparaginase has been a standard component of multiagent chemotherapy for the treatment of acute lymphoblastic leukemia (ALL), but side effects limit treatment compliance, 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 demonstrated positive efficacy and safety results in different studies in ALL, including 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. The positive results of its Phase 2b clinical trial in second line metastatic pancreatic cancer mark a first step 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 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 and enzyme replacement therapies.

*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).*

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## **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.