

## ERYTECH Reports First Half 2017 Financial Results and Provides Business Update

Conference call and webcast scheduled for  
Tuesday, September 12<sup>th</sup> at 02:30 pm CEST/08:30 am EDT

- Positive final results from Phase 2b study of eryaspase (GRASPA®) for the treatment of metastatic pancreatic cancer presented at the European Society for Medical Oncology (ESMO) 2017 Congress
- Meeting with the U.S. Food and Drug Administration (FDA) to seek guidance for further clinical development of eryaspase in pancreatic cancer planned
- Process for resubmission of European Marketing Authorization Application (MAA) for GRASPA for the treatment of relapsed or refractory acute lymphoblastic leukemia (ALL) initiated and on track for filing in October
- First patients enrolled in investigator-initiated study (NOPHO Study) in ALL
- Entered into research collaborations with Queen's University (Kingston, Canada) and the Fox Chase Cancer Center (FCCC) (Philadelphia, U.S.) to advance preclinical programs in metabolic diseases, and presented promising preclinical data of pipeline programs at several medical meetings during the first half of 2017
- U.S. team strengthened with hiring of U.S.-based Investor Relations, Regulatory Affairs and Strategic Marketing team members
- Successfully raised €70.5 million in gross proceeds in a private placement in April 2017
- Solid cash position of €88.5 million as of June 30, 2017

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**Lyon (France), September 11, 2017** – ERYTECH Pharma (Euronext Paris - ERYP), a clinical-stage biopharmaceutical company developing innovative therapies by encapsulating therapeutic drug substances inside red blood cells, today provided a business update and reported its financial results for the six-month period ended June 30, 2017.

*“The positive results of eryaspase in second-line metastatic pancreatic cancer have been highly significant for ERYTECH,”* commented Gil Beyen, ERYTECH’s Chief Executive Officer. *“Not only do these results open the door for eryaspase to become a potentially important therapy in the treatment of second-line metastatic pancreatic cancer, an area of high unmet medical need with very limited treatment options, they are also the basis for exploration of additional clinical development opportunities in this disease and in other solid tumors. Our capital raise in April significantly strengthened our balance sheet and enables us to build on these opportunities. The next key steps are meetings with the U.S. FDA, scheduled for October, and the Committee for Medicinal Products for Human Use, or CHMP, to be scheduled later in 2017, to discuss the development path and design of a potential Phase 3 study for eryaspase in pancreatic cancer. Concurrently, our preclinical teams are exploring other possible solid tumor indications in areas of high unmet medical need. In the meantime, we are preparing our MAA resubmission, including the supplementary data requested by the CHMP, for the potential EU approval of eryaspase for the treatment of relapsed or refractory ALL. We believe we have made substantial preclinical program advancements in oncology and metabolic diseases as part of our strategy to further expand our ERYCAPS technology.”*

## First Half and Recent Business Highlights

- ERYTECH presented positive full Phase 2b data from its 141-patient study evaluating eryaspase (GRASPA®) combined with chemotherapy in second-line metastatic pancreatic cancer at ESMO 2017. The study met its co-primary endpoints with respect to both overall survival (OS) and progression-free survival (PFS) in the entire patient population. Patients treated with eryaspase in combination with standard chemotherapy had a 40% reduction of risk of death compared to patients treated with chemotherapy alone (Hazard Ratio or HR of 0.60 (95% CI; 0.40, 0.88) (p=0.009). The HR for PFS was 0.59 (95% CI; 0.4, 0.89) (p=0.011). In the study, there was consistent clinical benefit across subgroups following treatment with eryaspase. The toxicity profile was similar between the two treatment arms. A summary of the results can be found in the press release issued last Friday, September 8, 2017. This data will also be discussed by the lead investigator for the study, Prof. Pascal Hammel, gastroenterologist-oncologist and head of oncology at Beaujon Hospital in Paris, at a company-sponsored investor and analyst event held during the ESMO 2017.
- The positive Phase 2b results from ERYTECH's metastatic pancreatic cancer study form the basis for the company's decision to pursue further development in this disease. Next steps include the design of a potential Phase 3 study in the U.S. and in Europe, which ERYTECH expects to discuss with the U.S. FDA in the coming weeks and the CHMP before the end of the year. These results also provide the basis for ERYTECH's decision to explore eryaspase as a treatment for other solid tumors, an area where non-encapsulated asparaginases have been investigated without success due to excessive toxicity.
- The clinical development of eryaspase (GRASPA®) in acute lymphoblastic leukemia (ALL) and in acute myeloid leukemia (AML) is progressing:
  - ERYTECH is preparing its MAA resubmission to the CHMP for the potential marketing authorization of GRASPA for the treatment of relapsed or refractory ALL in Europe, and intends to resubmit in October 2017 with supplemental data on comparability, immunogenicity and pharmacodynamics.
  - All patients in the third dose escalation cohort of ERYTECH's US Phase 1 study in first-line adult ALL have been treated. A decision on the recommended Phase 2 dosing is expected in the coming weeks, which will enable ERYTECH to begin planning a pivotal Phase 3 study.
  - An investigator-initiated study to assess eryaspase in patients with ALL was launched in Q2 of this year. The 30-patient, single arm, multi-center Phase 2 study is being conducted in seven Nordic and Baltic countries across 23 sites in collaboration with the Nordic Society of Pediatric Hematology and Oncology (NOPHO) and aims to evaluate the pharmacokinetic and pharmacodynamic activity, safety and immunogenicity profile of eryaspase in combination with NOPHO's 2008 multi-agent chemotherapy protocol for ALL, administered as second-intention treatment for children or adult ALL patients (1 to 45 years of age) who have experienced allergic or silent inactivation to PEG-asparaginase. The first patients were recently enrolled in this study.
  - ERYTECH's ongoing Phase 2b study in elderly patients with newly diagnosed (AML) is on track for primary results by the end of this year.
- Promising preclinical data from multiple ERYCAPS pipeline programs have been presented at medical meetings during 1H 2017:
  - In January and April, ERYTECH presented preclinical data supporting its product candidate erymethionase, consisting of methionine-γ-lyase (MGL) encapsulated in red blood cells, as a potential effective strategy for targeting methionine-dependent cancers, at the 2017 ASCO GI Symposium and AACR Annual Meeting, respectively.
  - In March, ERYTECH presented encouraging preclinical data supporting its immunotherapy program, ERYMMUNE, at two medical meetings, the World ADOPT Summit 2017 and the 10th Symposium of Vaccinology. In these studies, antigens encapsulated in modified red blood cells were able to delay tumor growth by inducing efficient and antigen-specific immune responses.

- In September, ERYTECH presented promising preclinical data on its eryminase and erymethionase programs at the 13<sup>th</sup> Annual International Congress of Inborn Errors of Metabolism (ICIEM). The findings from the research on eryminase, consisting of arginine deiminase encapsulated in red blood cells, showed a decrease in arginine levels in a disease model of arginase-1 deficiency, supporting a potential treatment approach for hyperargininemia. This study was conducted in collaboration with Queen's University (Kingston, Canada). In the study, administration of erymethionase resulted in lower homocysteine levels, supporting a potential treatment approach for homocystinuria. This study was conducted through a research collaboration with the Fox Chase Cancer Center (FCCC). Both collaborations underscore ERYTECH's intent to expand the scope of its ERYCAPS platform technology beyond oncology in response to collaboration opportunities with companies active in the field of metabolic diseases and enzyme replacement therapies.
- In April, ERYTECH raised €70.5 million in gross proceeds in a private placement through the issuance of 3,000,000 new ordinary shares to institutional investors in the United States and Europe. ERYTECH continues to use the proceeds from this capital increase primarily to fund the further clinical development of its product candidates, including the launch of a prospective Phase 3 study in pancreatic cancer and evaluation of clinical development opportunities for eryaspase in other solid tumor indications. A portion of the proceeds will also be used for general corporate purposes, operational expenses and working capital.
- Over the past months, ERYTECH continued to strengthen its team, especially in the United States, through the appointments of:
  - *Naomi Eichenbaum as its Director of Investor Relations.* Based in New York, Naomi leads the development and implementation of ERYTECH's investor relations strategy by communicating operational objectives, growth prospects and performance to the global investment community. Naomi has over 10 years of experience in life sciences and spent the last three years in investor relations at the Trout Group.
  - *Charles (Chuck) Monahan as its Senior Director of Regulatory Affairs.* Chuck is a seasoned regulatory affairs executive with more than 15 years of experience at companies such as Millennium Pharmaceuticals, AVEO Pharmaceuticals, Transgene and Eleven Biotherapeutics. Reporting to ERYTECH's Chief Medical Officer, Chuck is in charge of the global regulatory affairs for ERYTECH.
  - *Dan Cole, Director of Strategic Marketing & Business Development.* Dan brings 15 years of direct experience in oncology strategic marketing, market access and corporate development from working at companies such as Synta Pharmaceuticals, Abraxis BioScience and Ligand Pharmaceuticals. He provides strategic insight for the development and commercialization of ERYTECH's pipeline programs and supports business development activities. He reports to Jean-Sébastien Cleiftie, ERYTECH's Chief Business Officer, who relocated to the Company's Cambridge, MA office.
  - *Florence Renart-Depontieu as Group Leader of Immuno-oncology.* Based in Lyon, France, Florence develops and leads ERYTECH's research programs in immunotherapy. Florence holds a Ph.D. in immunology, followed by a post-doctoral program, at the John Hopkins University (U.S.). Before ERYTECH, she worked for the Ludwig Institute (Belgium) and BliNK Biomedical.

## Financial Highlights

ERYTECH's key financial figures for the first six months of 2017, compared with the same period of the previous year, are summarized below:

**Key figures (in thousands of euros):**

	1H (6 months) 2017	1H (6 months) 2016	Variation
Revenues	0	0	0
Other income	1,788	2,403	(616)
<b>Total operating income</b>	<b>1,788</b>	<b>2,403</b>	<b>(616)</b>
Operating expenses:			
Research & development	(12,082)	(8,800)	(3,283)
General & administrative	(3,895)	(4,222)	327
<b>Total operating expenses</b>	<b>(15,977)</b>	<b>(13,022)</b>	<b>(2,955)</b>
<b>Operating loss</b>	<b>(14,189)</b>	<b>(10,618)</b>	<b>(3,571)</b>
Financial income	114	260	(146)
Income tax	(5)	9	(14)
<b>Net Loss</b>	<b>(14,081)</b>	<b>(10,349)</b>	<b>(3,731)</b>

Net loss for the first half of 2017 was €14.1 million, compared to net loss of €10.3 million for the same period of last year. The €3.7 million increase reflected the continued efforts to advance ERYTECH's preclinical and clinical development programs. The increase was mostly driven by higher service and contracting fees, related to the clinical and regulatory progress of product development projects. Personnel costs also increased in the 2017 period compared to 2016 following the staffing of key additional positions in the preclinical and clinical domains, to address the activity expansion both in Europe and in the United States. Other income decreased in the 2017 period as a result of reduced research and development tax credits and grants.

As of June 30, 2017, ERYTECH had cash and cash equivalents totaling €88.5 million, compared with €37.6 million on December 31, 2016.

Total cash and cash equivalents as of June 30, 2017 included net proceeds of approximately €65.2 million from the April 2017 capital raise. Excluding the impact of the April 2017 cash raise, net cash utilization in the first half of 2017 was €14.5 million and reflected, as with net loss of the period, the increased activity in product development and the strengthening of ERYTECH's operations to prepare for its next stage of development.

The financial results for the first half of 2017 are in line with ERYTECH's expectations and strategy for 2017 which focus on advancing the preclinical and clinical developments of its innovative treatments for pancreatic cancer, ALL, AML and other solid tumors in Europe and in the United States.

The financial report for the six months ending June 30, 2017, is available on ERYTECH's website via the link: <http://erytech.com/financial-info.html#view2>.

**Upcoming Milestones Expected over Next 12 Months**

- Resubmission of EU MAA for GRASPA for the treatment of relapsed or refractory ALL
- Meetings with FDA and CHMP to discuss next steps for development of eryaspase in pancreatic cancer
- Maximum tolerated dose (MTD) defined in U.S. Phase 1 adult ALL study, and meeting with FDA on further ALL development plan
- Results from EU Phase 2b AML study
- Potential launch of Phase 3 study in the U.S. and EU of eryaspase in pancreatic cancer
- Potential launch of Phase 3 study in first-line adult ALL
- Potential launch of Phase 1 study with erymethionase

### First Half 2017 Conference Call Details

Investors and analysts wishing to participate can access the call via the following teleconferencing numbers:

**France:** +33 172001510

**Germany:** +49 69222229031

**USA:** +1 6467224907

**Belgium:** +32 24029640

**UK:** +44 2030432440

**Switzerland:** +41 225809022

**The Netherlands:** +31 107138194

**Sweden:** +46 850334664

**Finland:** +358 942599700

**Spain:** +34 914142021

Confirmation Code: **54851684#**

The webcast can be followed live online via the link:

<http://www.anywhereconference.com?UserAudioMode=DATA&Name=&Conference=135310581&PIN=54851684>

Following the live call, a replay will be available for 90 days. To listen to the replay, please dial:

**France:** +33(0)1 72 00 15 00

**USA:** +1 877 64 23018

**UK:** +44(0) 2033679460

**Spain:** +34 917896320

Confirmation Code: **310581#**

Additionally, an archive of the webcast will be available on the “Webcast” section of the Company’s investor relations site.

### Next Financial Updates:

- Financial highlights for the 3rd quarter of 2017: November 13, 2017 (after market close), followed by a conference call and webcast on November 14, 2017 (2:30pm CET/8:30am ET)

### Upcoming Investor Conferences:

- Morgan Stanley Global Healthcare Conference, September 13, New York
- Jefferies Global Healthcare Conference, November 15-16, London
- Actionaria, November 23-24, Paris
- ODDO BHF Forum, January 11-12, 2018, Lyon
- Investor access event at the J.P. Morgan Healthcare Conference, January 8-11, 2018, San Francisco

### 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 multiagent 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).*

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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.