

## ERYTECH Announces Collaboration with Queen's University to Advance its Product Candidate for Rare Metabolic Disorders

**Lyon (France), July 12, 2017** – ERYTECH Pharma (Euronext Paris - ERYP), a French clinical-stage biopharmaceutical company developing innovative therapies for rare forms of cancer and orphan diseases, today announced that it has entered into a research collaboration with Queen's University in Canada to advance the preclinical development of ERYTECH's eryminase program specifically for the treatment of arginase-1 deficiency, a rare and severe metabolic disorder related to arginine metabolism. The collaboration will leverage the expertise of Queen's University and ERYTECH's ERYCAPS platform technology with the goal of generating *in vivo* proof-of-concept data in an arginase-1 deficiency animal model.

Arginase-1 deficiency is a rare, inherited disorder of the urea cycle caused by a mutation in the arginase-1 gene, resulting in the accumulation of toxic levels of the amino acid arginine in the blood. Symptoms generally appear in early infancy and include intellectual disability, non-ambulatory muscle stiffness and seizures. It is a debilitating, progressive disease with very limited treatment options currently available.

The collaboration between ERYTECH and Queen's University aims to demonstrate the potential of ERYTECH's eryminase to lower arginine in the inducible arginase-1 deficiency mouse model developed by the laboratory of Prof. Colin Funk of Queen's University. Eryminase, a product candidate being developed by ERYTECH, consists of an arginine deiminase enzyme encapsulated in red blood cells using ERYTECH's proprietary ERYCAPS platform technology. ERYTECH believes the encapsulation of the therapeutic enzymes in the red blood cells can provide effective, long-acting therapeutic activity with reduced toxicity.

**Prof. Colin Funk**, PhD, Queen's University, commented, *"Arginase-1 deficiency is a severe, rare disorder affecting a biochemical pathway that disposes of toxic ammonia. Normally, our bodies are very efficient at removing any ammonia that accumulates after eating a protein-rich meal. However, in patients with arginase-1 deficiency, the ammonia is "partially detoxified" leading to a large accumulation of the amino acid arginine in the patient's blood and brain. ERYTECH's product candidate eryminase aims to reduce the level of arginine in blood and thus, has significant potential to reduce negative consequences of this disorder. We look forward to working with ERYTECH to advance their preclinical program."*

**Dr. Alexander Scheer**, PhD, Chief Scientific Officer of ERYTECH, added, *"This is our second collaboration in the field of rare metabolic diseases that underscores the scope of our platform and its applicability to highly specialized and rare conditions beyond oncology. We are very pleased to enter this collaboration with Queen's University and look forward to working closely on this important program with Dr. Funk who specializes in research related to urea cycle disorders."*

### About Queen's University

Queen's University is one of the leading research-intensive institutions in Canada. The mission is to advance research excellence, leadership and innovation, as well as enhance Queen's impact at a national and international level. Through undertaking leading-edge research, Queen's is addressing many of the world's greatest challenges, and developing innovative ideas and technological advances brought about by discoveries in a variety of disciplines. Queen's University is a member of the U15 Group of Canadian Research Universities.

## About ERYTECH: [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 treatment of blood cancers, including acute lymphoblastic leukemia (ALL) and acute myeloid leukemia (AML), by depriving tumors of nutrients necessary for their survival. ERYTECH's lead product candidate, eryaspase, also known under the trade name GRASPA<sup>®</sup>, reported positive efficacy and safety results from its completed Phase 2/3 pivotal clinical trial in Europe in children and adults with relapsed or refractory ALL. A Phase 1 clinical study of eryaspase is ongoing in the United States in adults with newly diagnosed ALL, and a Phase 2b clinical study in Europe in elderly patients with newly diagnosed AML, each in combination with chemotherapy.

The company believes that eryaspase also has potential as a treatment approach in solid tumors. ERYTECH has successfully completed Phase 2b clinical trial in France evaluating eryaspase in patients with second-line metastatic pancreatic cancer.

Eryaspase 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, from circulating blood plasma.

The EMA and the U.S. Food and Drug Administration (FDA) have granted orphan drug designations for eryaspase (GRASPA) for the treatment of ALL, AML and pancreatic cancer. 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<sup>®</sup> brand name.

In addition to eryaspase, ERYTECH is developing other product candidates targeting cancer metabolism: erymethionase and eryminase, respectively methionine-γ-lyase and arginine-deiminase encapsulated in red blood cells. ERYTECH is exploring furthermore exploring the use of its platform in immune-oncology (ERYMMUNE) and enzyme 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.