

## ERYTECH business update and financial results for the first half of 2013

- Strong operational performance with all clinical programs on track
- Successful IPO and rigorous cash management securing a solid cash position
- Solid news flow ahead

---

**Lyon (France), August 28, 2013** – ERYTECH (NYSE Euronext Paris: FR0011471135 - ERYP), a French biopharmaceutical company that develops innovative treatments for acute leukemia and other oncology indications with unmet medical needs, provides a business update and reports its financial results for the period ending June 30, 2013.

### Business Highlights

- Patient enrollment in the Phase III study in Acute Lymphoblastic Leukemia (ALL) on track for results in Q3 2014
- Orphan drug designation granted to GRASPA® in Acute Myeloid Leukemia (AML)
- Rapid take-off and fast enrollment in the Phase IIb study with GRASPA® in AML
- FDA IND clearance to start clinical development in the USA
- IP portfolio strengthened
- Preclinical development programs in solid tumors and other orphan indications progressing well

### Financial Highlights

- € 16.7 million raised in successful IPO
- Operating result maintained notwithstanding the increased activity level
- Strong cash balance of € 18.5 million

### Upcoming Milestones

- Complete enrollment of the Phase III study in ALL in Europe
- Enrollment of the first patients in the Phase Ib study in ALL in the US
- Safety update of the Phase IIb study in AML
- Launch of a Phase II study in solid tumors

*“The first half of 2013 has clearly been a crucial and successful period for ERYTECH. Not only did we achieve a number of critical business milestones, we also realized a successful IPO, securing a strong cash and financial position.”* comments Gil Beyen, Chairman and CEO of ERYTECH. *“We are consistently delivering on the objectives we set at the time of our IPO, and have built the basis for the Company’s next phase of development: our clinical programs in Europe are fully on track, the partnership with Orphan Europe/Recordati is working well, we are launching clinical development in the United States and we have made substantial headway in our R&D activities to broaden our scope to other indications of high unmet medical needs. With all of this, ERYTECH is building a clear strategic value in the field of orphan oncology. In the second semester of 2013 we expect to realize a number of important objectives towards achieving this goal.”*

## Business Update

### Patient enrollment in the Phase III study in ALL on track for results in Q3 2014

The enrollment of patients in ERYTECH's pivotal Phase III study in Acute Lymphoblastic Leukemia (ALL) is going well and nearing completion. The study is comparing GRASPA® to native asparaginase in a randomized controlled and multicenter clinical trial on 80 children and adults suffering from relapsing or refractory ALL. The study was launched in 2009 as a Phase II/III study with an adaptive design protocol. During the first quarter of this year an external Data Safety Monitoring Board (DSMB) independently reviewed the data concerning the first 60 patients and recommended the transition to Phase III and to continue the study without changes to the protocol. Full results of this pivotal study are expected in Q3 14.

### Orphan drug designation granted to GRASPA® in AML

In February 2013, the European Medicines Agency (EMA) granted GRASPA® orphan drug status for the treatment of Acute Myeloid Leukemia (AML). This orphan drug status provides certain advantages for the sponsor, such as reduced procedural costs and ten years of commercial exclusivity.

With about 34,000 new patients per year in Europe and the US, AML is the most common type of acute leukemia. Affecting mainly the adult and senior patient population that often cannot tolerate existing forms of asparaginase products, AML represents one of the highest mortality rates among all types of cancers and a major unmet medical need.

With the orphan drug designation in AML, ERYTECH now benefits from 4 orphan drug designations in Europe and 2 in the U.S. As well as AML and ALL, the company also has orphan drug status in Pancreatic Carcinoma and Sickle Cell Disease.

### Rapid take-off and fast enrollment in the Phase IIb study with GRASPA® in AML

In March 2013, ERYTECH initiated a multicentre, open, randomized, controlled Phase IIb trial evaluating the efficacy and tolerability of GRASPA® in the treatment of newly diagnosed AML patients, over 65 years old, unfit for intensive chemotherapy.

By the end of June, all 21 French investigation sites had been authorized to participate in the study. With 16 of these sites initiated and opened so far, 26 out of a total of 123 patients have already been enrolled in the study. The current pace of inclusion should enable ERYTECH to recruit the last patient in the study before the scheduled date of the end of 2014. Simultaneously to the patient recruitments realized in France, the opening of other specialized centers in different European countries is planned with a view to internationalizing the study and further accelerating patient enrollment.

The study is being performed in collaboration with Orphan Europe (Recordati Group), ERYTECH's partner for the commercialization of GRASPA® in ALL and AML in 38 European countries, under a licensing and distribution agreement that was signed at the end of 2012.

### FDA IND clearance to start clinical development in the USA

Also in March 2013, ERYTECH received clearance for its Investigational New Drug (IND) Application from the United States Food and Drug Administration (FDA) to initiate a Phase I clinical trial of GRASPA®/ERYASP<sup>1</sup> in patients 40 years old or older with newly-diagnosed Acute Lymphoblastic Leukemia (ALL).

Prof. Richard A. Larson, Director of the Hematological Malignancies Clinical Research Program at the University of Chicago and former Chairman of the Leukemia Committee of the Cancer and Leukemia Group B (CALGB), is the principal investigator of the study, and two additional academic medical centers will be participating.

The launch of clinical development in the United States has been anticipated and well prepared by ERYTECH. In 2010, ERYTECH entered into collaboration with the American Red Cross in Philadelphia, securing the supply of red blood cells as well as a fully operational manufacturing facility where ERYASP will be manufactured for the clinical trials.

---

<sup>1</sup> ERYASP is the name used for GRASPA® outside Europe and for indications other than ALL and AML. The GRASPA® brand name has been licensed to Orphan Europe for the commercialization of the product in ALL and AML in Europe.

## IP portfolio strengthened

During the first half of 2013, two new patent applications were filed (one “Process” patent and one “Product” patent) and four existing patents were granted in new countries.

On June 30, 2013 ERYTECH was holder of 12 patent families, covering its technology platform and applications thereof in and outside oncology, and of an exclusive license from the National Institutes of Health (USA), covering a diagnostic method to predict the efficacy of L-asparaginase.

## Preclinical programs in solid tumors and other orphan indications progressing well

Simultaneously to pushing forward the clinical development of GRASPA®, solid headway has been made in preclinical development in the area of solid tumors and other indications:

### *Solid tumors*

- With a view to launching a Phase II study with ERYASP in solid tumors, different potential indications have been evaluated for their sensitivity to asparaginase;
- In the meantime, the Oséo co-funded TEDAC program is progressing well. The TEDAC program provides up to €7 million of government funding over a period of 7 years in the form of subsidies and innovation credits to help broaden the use of the technology to solid tumors. Different therapeutic enzymes (including asparaginase) are being tested on their tumor starvation ability.

### *Other indications*

Simultaneously to the priority development programs in oncology, ERYTECH is pursuing preclinical development work to investigate the enlargement of the use of its innovative technology platform, allowing the entrapment of therapeutic compounds in red blood cells, in other indications. During the first half of 2013 the results of two programs have been published in peer reviewed journals:

- Resulting from our collaboration with GENZYME regarding the entrapment of the enzyme used for the treatment of PKU (phenylketonuria), a publication, titled “*Erythrocytes encapsulated with phenylalanine hydroxylase exhibit improved pharmacokinetics and lowered plasma phenylalanine levels in normal mice*”, was published in *Molecular Genetics and Metabolism* in July 2013;
- Another application outside oncology of ERYTECH’s proprietary technology platform is the induction of tolerance to certain immunogenic proteins. The proof of principle “*Red blood cells as innovative antigen carrier to induce specific immune tolerance*” was published in *International Journal of Pharmaceutics* (443 (2013) 39– 49). This work was selected to be presented at the 15<sup>th</sup> International Congress of Immunology in Milano, Italy on August 27, 2013 via an oral presentation.

ERYTECH’s Scientific and Medical Advisory Board met on June 28, 2013 in Lyon. Clinicians and scientists from Europe and the United States discussed the Company’s development programs.

## Financial Update

### € 16.7 million raised via a successful IPO on NYSE Euronext

On April 30, 2013, ERYTECH announced the success of its IPO in compartment C of the NYSE Euronext regulated market in Paris, raising €16.7 million, more than the target amount of €15 million, with a further €1 million subscribed through debt compensation.

In addition to the €17.7 million subscribed to via the IPO, a conversion of convertible bonds for a total of €10 million - including the acquisition of a stake by Recordati - also took place, bringing the total size of the capital increase operation to €27.8 million.

### Operating result maintained notwithstanding an increased activity level

The net loss for the first six months of 2013 amounted to €4.1 million. This represents a decrease of approximately €0.1 million (or 3%) compared with the same period last year. This decrease is essentially the result of a €0.5 million increase in other income (subsidies and tax credits) offset by a €0.4 million increase in operating costs. This increase is in line with the increase in the level of activity, mainly in R&D and clinical development.

Total Research and Development expenses, including the cost of clinical studies and expenses related to intellectual property management, for the first half of 2013 increased by €0.5 million to €2.3 million compared with €1.9 million for the first half of 2012.

Selling, general and administrative expenses decreased by €0.1 million, notwithstanding our listing on NYSE Euronext Paris, reflecting the strong cash management the company is adopting.

The net use of cash in operating and investing activities for the first six months of 2013 was €4.2 million, in line with management expectations.

### **Solid cash balance of € 18.5 million**

As a result of the above, ERYTECH has a strong balance sheet with cash and cash equivalents of €18.5 million at end June 2012 compared with €7.9 million on December 31, 2012.

The table below summarizes ERYTECH's key financial figures for the first half of 2013 compared with the same period of the previous year.

	<b>Period to June 30</b>	
	<b>2013</b>	<b>2012</b>
Sales	0	0
Other income	858	342
<b>Operating income</b>	<b>858</b>	<b>342</b>
R&D expenses	1,157	857
Clinical trial costs	992	795
IP expenses	198	209
SG&A expenses	1,450	1,528
Total operating costs	3,797	3,388
<b>Operating result</b>	<b>-2,939</b>	<b>-3,046</b>
Financial result	-1,123	-1,131
Tax	6	-1
<b>Net Result</b>	<b>-4,056</b>	<b>-4,178</b>

The full financial report for the first semester of 2013, as approved by the Board of Directors on August 28, 2013, is available on ERYTECH's website ([www.erytech.com](http://www.erytech.com)). The report has been subject to a limited review procedure by the company's statutory auditors.

### **Next financial updates:**

- Financial highlights for the 3<sup>rd</sup> quarter of 2013: Tuesday, 5 November 2013 (after market)

### **Upcoming presentations at investor conferences:**

- Sachs Annual Biotech in Europe Investor Forum, September 30 and October 1, 2013 in Zurich
- CF& B Midcap Event, October 2 and 3, 2013 in Paris

## About ERYTECH: [www.erytech.com](http://www.erytech.com)

Created in Lyon in 2004, ERYTECH is a French biopharmaceutical company providing new prospects for cancer patients, particularly those with acute leukemia. 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, representing a market opportunity of more than EUR 1 billion. By encapsulating the asparaginase enzyme in red blood cells, ERYTECH has developed GRASPA<sup>®</sup>, 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. GRASPA<sup>®</sup> is currently completing Phase III clinical development in Acute Lymphoblastic Leukemia (ALL) and is in Phase IIb clinical trial in Acute Myeloid Leukemia (AML). ERYTECH has concluded distribution partnership agreements for Europe with Orphan Europe (Recordati Group), and with TEVA for Israel. In the United States, ERYTECH is launching a Phase Ib clinical trial in ALL, after having received approval from the US FDA. 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 NYSE 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](http://www.amf-france.org)), also available on our 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 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.

## CONTACTS

**ERYTECH**  
**Gil Beyen**  
*Chairman and CEO*  
**Pierre-Olivier Goineau**  
*Vice President and COO*  
Tel: +33 4 78 74 44 38  
[investors@erytech.com](mailto:investors@erytech.com)

*NewCap.*  
**Julien Perez / Emmanuel Huynh**  
Investor and press relations  
Tel: +33 1 44 71 98 52  
[erytech@newcap.fr](mailto:erytech@newcap.fr)

