

## ERYTECH provides business update and financial results for the full year 2013

- Strong operational performance with clinical programs on track
- Solid cash position
- Important news flow ahead

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**Lyon (France), April 29, 2014** – ERYTECH (Euronext Paris: FR0011471135 - ERYP), the French biopharmaceutical company that develops innovative ‘tumor starvation’ 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 December 31, 2013.

### Business Highlights

- Phase III study in Acute Lymphoblastic Leukemia (ALL) fully enrolled and on track for results in Q3 2014
- Orphan drug designation granted to GRASPA®/ERY-ASP<sup>1</sup> in Acute Myeloid Leukemia (AML) in Europe and the USA
- Launch of a Phase IIb study in AML, recruitment on track and positive DSMB on first 30 patients
- FDA IND clearance to start clinical development in the USA and ; centers open for patient enrollment
- Launching Phase II trial in pancreatic cancer
- New oncology product candidate added and preclinical development programs progressing well
- IP portfolio strengthened and core patent granted in the US with 4 years extension of term

### Financial Highlights

- € 16.7 million raised in successful IPO on NYSE Euronext Paris
- Operating costs maintained notwithstanding the increased activity level
- Strong cash balance of € 15.1 million on December 31, 2013

### Upcoming Milestones

- Enrollment of the first patients in the Phase Ib study in ALL in the USA
- Second DSMB safety update of the Phase IIb study in AML
- Results of European Phase III study in ALL
- DSMB update on Phase II pancreas cancer study

*“2013 has been a pivotal year for ERYTECH. Not only did we achieve a number of critical business milestones, we also realized a successful IPO, securing a strong financial position.”* comments Gil Beyen, Chairman and CEO of ERYTECH. *“We have advanced our clinical trials in acute leukemia according to plan and we have made substantial headway in broadening our scope in pancreas cancer and advancing our preclinical pipeline with new products in oncology. ERYTECH is delivering on its IPO promise to build a strategic value in the field of orphan oncology.”*

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<sup>1</sup> ERY-ASP 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.

## **Business Update**

### **Phase III study in ALL fully enrolled and on track for results in Q3 2014**

In August 2013, ERYTECH completed patient enrollment in its pivotal Phase III study in Acute Lymphoblastic Leukemia (ALL). 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 the 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 2014.

### **Orphan drug designation granted to GRASPA®/ERY-ASP in AML in Europe and the USA**

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.

About a year later, in March 2014, ERYTECH also received Orphan Drug Designation by the FDA in Acute Myeloid Leukemia. In the USA, Orphan Drug Designation (ODD) is generally granted to drugs or biologics intended for treatment of rare diseases and disorders of high unmet medical need, affecting fewer than 200,000 people. This designation conveys special incentives to the sponsor, including seven years of US market exclusivity for the drug or biologic upon FDA approval, a prescription drug user fee waiver, and certain tax credits.

The latest ODD is the seventh for ERYTECH. GRASPA/ERY-ASP now benefits from ODD in all three of its lead indications, ALL, AML and pancreas cancer, both in Europe and the USA.

### **Launch of a Phase IIb study in AML, recruitment on track and positive DSMB on first 30 patients**

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.

AML, the most common form of acute leukemia, affects about 34,000 new patients per year in the US and Europe, mainly adults and older patients, for which few therapeutic options are available today.

The rationale for using asparaginase in AML is strong. However, as the median of age of AML patients is about 70 years old, a large majority of them are too fragile to tolerate the current asparaginase formulations. Thanks to its better safety profile, GRASPA® aims to make asparaginase available also to these patients.

Today, close to half of a total of 123 patients have been enrolled in the study in 13 active French centers. Over the past months the company received authorizations for its Phase IIb trial in Spain, Finland and Italy. Germany is pending. The opening of centers in these countries will internationalize the study and further accelerate 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, centers open for patient recruitment**

Also in 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 ERY-ASP in 12 to 18 patients, 40 years old or older, with newly-diagnosed ALL.

Three participating centers, The University of Chicago, Duke Medical Center and Ohio State University have been fully authorized and are ready to enroll patients. 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.

## **Launching Phase II trial in pancreatic cancer**

As part of its strategy to broaden the scope of its lead product ERY-ASP, ERYTECH has retained pancreatic cancer, a very aggressive form of cancer with few treatment options, as the first indication for the product in solid tumors.

Having already successfully completed a Phase I study in late stage pancreas cancer, in which the tolerability of ERY-ASP has been confirmed in this very fragile patient population, ERYTECH decided to continue the development in solid tumors by performing a Phase II study in second line treatment of patients with progressive metastatic pancreas cancer.

Scientific advice had been obtained at the end of 2013 from the European Medicines Agency (EMA) and a clinical trial application (CTA) was subsequently submitted to the ANSM, the French authority for drug safety.

In a study of about 100 patients, ERY-ASP in addition to the best standard of care would be compared to the best standard of care alone in a 2-to 1 randomization. The primary endpoint would be progression free survival (PFS) at 4 months after start of treatment. Patients will be stratified according to the expression of asparaginase synthetase (ASNS) of their primary tumor. Patient enrollment is expected to start in Q2 2014.

Professor Pascal Hammel, gastro-enterologist specialized in digestive oncology at Hôpital Beaujon (Clichy-Paris, France), will be the primary investigator of the study.

## **New product candidate added and preclinical development programs progressing well**

Simultaneously solid progress has been made in the preclinical development in the field of oncology:

- The work done in the government co-funded TEDAC program to broaden the use of ERYTECH's encapsulation technology to other enzymes has led to the identification of a promising new product candidate, ERY-MET, consisting of methionine- $\gamma$ -lyase (MGL) encapsulated inside red blood cells. With its proprietary encapsulation technology, ERYTECH has succeeded the encapsulation of MGL inside red blood cells with a good stability. The in vivo half-life of the encapsulated enzyme was extended to multiple days from a few hours for the free form. Based on these promising preclinical results, the company will continue with the preclinical development and progress it to the stage of clinical testing. The industrial scale-up of the manufacturing will be initiated in the coming months to enable a first-in-man Phase I study in 2015;
- In view to potentially launching additional studies with ERY-ASP in solid tumors, different potential indications have been evaluated for their sensitivity to asparaginase: next to pancreas cancer, opportunities are being investigated in Non Hodgkin lymphoma, multiple myeloma, liver cancer, bladder cancer and ovarian cancer;
- ERYTECH is also investigating its encapsulation technology in the field of immuno-therapy. Its proprietary technology in this field (Vaccin'ERY) consists in encapsulating specific antigens that can trigger an immunological response against cancer cells and steering these antigen-loaded red blood cells antigen presenting cells in the spleen. Promising results of this program have been published in "Vaccine" and the "Journal of Immunotherapy".

## **IP portfolio strengthened and core patent granted with four years of additional term in the USA**

In 2013, two new patent applications were filed (one "Process" patent and one "Product" patent) and five patents were granted in new countries.

At the end of 2013, the US patent office (USPTO) delivered its final notification of allowance for the granting of ERYTECH's core patent in the USA in which the term of exclusivity has been extended with almost four years. This leads to patent protection until April 2029, a term that can be further extended to 2034 on the basis of the patent term extension that may be available based on future marketing authorization. The patent was filed in 2004 and has in the meantime been granted in Europe, Australia, China, Hong-Kong, Japan and now also the USA. This patent is the core protection of ERYTECH's technology and products, including the GRASPA®/ERY-ASP product in liquid and solid tumors.

ERYTECH is today holder of 13 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.

## Financial Update

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

On April 30, 2013, ERYTECH announced the success of its IPO in compartment C of the 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 raised in 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 costs maintained notwithstanding the increased activity level

The net loss for the full year 2013 amounted to €8.1 million. This represents an increase of approximately €6.0 million compared with the same period last year. This increase is essentially the result of the €5.0 million upfront payment made by Orphan Europe (Recordati) late 2012 for the exclusive distribution agreement of Grasp<sup>a</sup>® in ALL and AML in Europe. The remaining difference of €1.0 million is essentially the result of an increase in other income (mainly Research Tax Credits) by €1.1 million, offset by a €2.1 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 R&D expenses, including the cost of clinical studies and expenses related to intellectual property management, for 2013 increased by €1.9 million to €5.3 million compared to €3.5 million for 2012. This reflects notably the increased number of clinical studies and the R&D activities linked to ERY-ASP and the TEDAC program.

Selling, general and administrative expenses increased by €0.2 million, notwithstanding our listing on Euronext Paris, reflecting the strong cash management the company is adopting.

When making abstraction of the extraordinary income of €5.0 million in 2012 and the extraordinary cost of €0.6 million related to the IPO in 2013, the recurrent net loss would have been €7.5 million in 2013 compared to €7.2 million in 2012.

### Solid cash balance of € 15.1 million

As a result of the above, ERYTECH has a strong balance sheet with cash and cash equivalents of €15.1 million at end of 2013 compared with €7.9 million on December 31, 2012.

The table below summarizes ERYTECH's key financial figures for 2013 compared with the previous year.

<i>Key figures (in thousands of euros): IFRS</i>		
	<b>2013</b>	<b>2012</b>
Sales	0	0
Other income	1,802	5,737
<b>Operating income</b>	<b>1,802</b>	<b>5,737</b>
R&D expenses	2,503	1,623
Clinical trial costs	2,462	1,393
IP expenses	363	445
SG&A expenses	3,587	3,436
Other operating costs	-28	-86
Total operating costs	8,887	6,811
<b>Operating result</b>	<b>-7,085</b>	<b>-1,074</b>
Financial result	-1,100	-1,090
Taxes	40	-8
<b>Net result</b>	<b>-8,144</b>	<b>-2,172</b>

The full financial report for the year ending December 31, 2013, as approved by the Board of Directors on April 25, 2014, is available on ERYTECH's website ([www.erytech.com](http://www.erytech.com)). The report has been subject to a full review procedure by the company's statutory auditors.

### **Next financial updates:**

- Financial highlights for the 1<sup>st</sup> quarter of 2014: Thursday, 15 May 2014 (after market)

### **Upcoming participations at investor conferences:**

- Goldman Sachs Healthcare Investment Forum, May 6 -7 in London
- Gilbert Dupont, Midcap Healthcare Forum, May 13 in Paris
- BioEquity Europe, May 21-22 in Amsterdam
- Jefferies Global Healthcare Conference, June 2-5 in New York
- French Life Sciences Day, June 26 in New York

### **About ERYTECH and ERY-ASP/GRASPA®: [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 and selected solid tumors.

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. By encapsulating the asparaginase enzyme in red blood cells, ERYTECH has developed ERY-ASP/GRASPA®, 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, representing a market opportunity of more than EUR 1 billion. ERY-ASP/GRASPA® is currently completing Phase III clinical development in Acute Lymphoblastic Leukemia (ALL) and is in Phase IIb clinical trial in Acute Myeloid Leukemia (AML). The product also received FDA clearance to start clinical development in ALL in the USA. ERYTECH has concluded distribution partnership agreements for Europe with Orphan Europe (Recordati Group), and with TEVA for Israel.

The company is also developing other indications in solid tumors and certain orphan indications outside oncology. ERYTECH is launching a Phase II study in pancreas cancer in Europe. The company holds orphan drug designation for ERY-ASP/GRASPA® in ALL, AML and pancreas cancer in Europe and the USA. ERYTECH has its own GMP-approved and operational manufacturing sites in Lyon (France) and Philadelphia (USA).

*ERYTECH is listed on NYSE Euronext regulated market in Paris. (ISIN code: FR0011471135, ticker: ERYP) and is part of the CAC All Shares, CAC Healthcare, CAC Pharma & Bio, CAC Small, CAC Mid&Small, CAC All Tradable and Next Biotech indexes. ERYTECH shares are eligible to PEA-PME (French share savings plan for SMEs).*

### **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.

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