

## ERYTECH business update and financial results for the first half of 2014

- Clinical trials on track and two new studies started
- Shareholder base further internationalized
- Solid cash balance

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**Lyon (France), September 2<sup>nd</sup>, 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 June 30, 2014.

### Business Highlights

- Phase III clinical study in Acute Lymphoblastic Leukemia (ALL) on track for results early Q4
- International sites opened in Phase IIb Acute Myeloid Leukemia (AML) study and positive Data Safety Monitoring Board (DSMB) assessment after first 60 patients
- Phase II study launched in pancreatic cancer; first patient enrolled
- Phase I study in ALL launched in the USA; first patient enrolled
- Orphan drug designation granted to ERY-ASP<sup>1</sup> in AML in the USA
- New oncology product candidate added (ERY-MET) and preclinical development programs on track
- IP portfolio reinforced
- Board of directors strengthened with two new independent members

### Financial Highlights

- Shareholder base further internationalized after successful share replacement operation
- Net loss reduced and operating costs maintained notwithstanding increased activity level
- Cash balance of € 12.3<sup>2</sup> million on June 30, 2014

### Upcoming Milestones

- Results of European Phase III ALL study
- DSMB analysis for safety and futility in Phase IIb AML study
- First DSMB update on Phase II pancreas cancer study

*“ERYTECH has made important progress during the first half of 2014. We have advanced our clinical trials in acute leukemia according to plan and we have made substantial headway in broadening our scope in solid tumors. We are also advancing our preclinical pipeline with new products in oncology. In addition we were able to strengthen our shareholder structure, with additional specialized investors in the US and Europe, as well as our board, with two new independent directors.”*, commented Gil Beyen, Chairman and Chief Executive Officer of ERYTECH.

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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.

<sup>2</sup> Excluding € 0.2 million available in the liquidity plan

## **Business Update**

### **Phase III clinical study in ALL on track for results early Q4**

In August 2013, ERYTECH completed patient enrollment in its pivotal Phase III study in Acute Lymphoblastic Leukemia (ALL). The last one-year follow up visit took place in August. Data base cleaning is ongoing and ERYTECH expects to communicate top-line results early Q4. The study is comparing GRASPA® with native asparaginase in a randomized, controlled, multicenter clinical trial with 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.

### **International sites opened in Phase IIb AML study and positive DSMB assessment after first 60 patients**

In March 2013, ERYTECH initiated a multicenter, open, randomized, controlled Phase IIb trial evaluating the efficacy and tolerability of GRASPA® in the treatment of newly diagnosed Acute Myeloid Leukemia (AML) patients, over 65 years of age, unfit for intensive chemotherapy.

Today, more than half of a total of 123 patients have been enrolled in the study in 15 active centers in France. The company has recently received regulatory authorizations for this trial in Spain, Finland, Germany and Italy, and the first 4 sites outside France have been initiated. The opening of these European centers will internationalize the study and further accelerate patient enrollment.

Last week the company announced that an independent Data and Safety Monitoring Board (DSMB) completed its second safety assessment of the study and unanimously recommended continuation of the trial without modification. This second DSMB assessment was based on a pre-planned safety analysis on the first 60 patients included in the study and with a minimum of 1 month follow-up. A first DSMB assessment took place at the end of 2013 when 30 patients had been treated in the study. The next step will be another DSMB analysis, this time for safety and futility, when 60 patients will have experienced an event in the study. This analysis is foreseen by the end of this year.

### **Phase II trial launched in pancreatic cancer; first patient enrolled**

After the completion of a Phase I study in late stage pancreas cancer, in which the tolerability of ERY-ASP was found acceptable in this fragile patient population, ERYTECH launched a Phase II study in 2014 in second line treatment of patients with progressive metastatic pancreas cancer. Clinical trial authorization was received in April by the ANSM, the French authority for drug safety. The first patient was enrolled in July 2014.

The study is planned for 90 patients whereby ERY-ASP in addition to the standard of care will be compared to the standard of care alone in a 2-to-1 randomization. The primary endpoint is progression-free survival (PFS) at 4 months. Professor Pascal Hammel, gastro-enterologist specialized in digestive oncology at Hôpital Beaujon (Clichy-Paris, France), is the primary investigator of the study.

### **Phase I/II study in ALL launched in the USA; first patient enrolled**

Having received Investigational New Drug (IND) clearance from the US Food and Drug Administration (FDA) in 2013 to start a Phase I clinical trial of ERY-ASP in ALL, ERYTECH has launched a dose escalating study in 12 to 18 ALL patients earlier this year. Three centers are currently open for patient recruitment (The University of Chicago, Duke University Medical Center and Ohio State University) and the first patient was recently enrolled and treated.

The investigational drug has been produced at ERYTECH's manufacturing facility in Philadelphia. Through a manufacturing agreement with the American Red Cross, this facility is operational for GMP production of clinical batches. Professor Larson, Director of the Hematological Malignancies Clinical Research Program at the University of Chicago is the principal investigator of the study.

## **Orphan drug designation granted to ERY-ASP in AML in the USA**

In March 2014, FDA granted Orphan Drug Designation (ODD) to ERY-ASP in AML. In the USA, an 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 contains additional incentives to the sponsor, including seven years of US market exclusivity for the drug after regulatory approval.

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.

## **New product candidate added and preclinical development programs on track**

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 drug candidate, ERY-MET. ERY-MET consists of methionine- $\gamma$ -lyase (MGL) encapsulated inside red blood cells. Using its proprietary encapsulation technology, ERYTECH has succeeded the encapsulation of MGL with a good stability and an extended half-life. Based on these promising preclinical results, the company will continue with the preclinical development to the stage of clinical trials. The industrial scale-up of the manufacturing will be initiated to enable a first-in-man Phase I study by the end of 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.

## **IP portfolio reinforced**

During the first half of 2014, ERYTECH received notice of allowance from the European Patent Office of a key patent covering its lead product ERY-ASP for the treatment of pancreas cancer. The patent entitled "Medicament for the Treatment of Cancer of the Pancreas" was already granted in Australia, Israel and Singapore.

ERYTECH's core process patent was also recently granted in India. This patent entitled "Lysis/Resealing Process for Preparing Erythrocytes" was already granted in Europe, US, Japan, China, Hong-Kong, Australia and South-Korea.

As of mid-2014 ERYTECH was the holder of 13 patent families, covering the technology platform and applications thereof in and outside oncology, as well as an exclusive license from the National Institutes of Health (USA), covering a diagnostic method to predict the efficacy of L-asparaginase.

## **Board of Directors strengthened with two new independent members**

At the General Shareholder's meeting in June 2014, two new independent members have been appointed to the Board of Directors:

- Martine George, M.D. is an experienced, US based clinical research, medical affairs and regulatory affairs executive, both in large and small oncology companies. Until recently, Dr George was Vice President Global Medical Affairs, Oncology at Pfizer in New York. Her previous functions before Pfizer included Chief Medical Officer at GPC Biotech in Princeton and Head of oncology at Johnson & Johnson in New Jersey. Martine is a board certified Medical Oncologist and Gynecologist, trained in France and Montréal. She started her career as a clinician as Service Chief at Institut Gustave Roussy in France and as Visiting Professor at Memorial Sloan Kettering Cancer Center in New York.
- Hilde Windels has over 20 years' experience in corporate finance, capital markets and strategic initiatives. She is the Chief Financial officer of Biocartis, a molecular diagnostics and immunodiagnostics company based in Belgium and Switzerland. Before Biocartis, Hilde was Devgen's CFO (Euronext: DEVG) from 1999 until the end of 2008 and member of Devgen's board from 2001 until the end of 2008. From early 2009 to mid 2011, she worked as independent CFO for a few private biotechnology companies, and she was on the board of MDX Health (Euronext: MDXH) from June 2010 until end of August 2011.

Kurma Life Science Ventures, represented by Mrs Vanessa Malier and representing Idinvest on the ERYTECH board has resigned from the Board. The Board of Directors wish to thank Idinvest, Kurma Life Sciences and Vanessa Malier for their contributions to the company.

## Financial Update

### Shareholder base further internationalized after successful share replacement operation

In February 2014, certain of the historical institutional investors of ERYTECH have sold part of their holdings to new investors in a successful share replacement operation. Under the deal, 17.5% of its capital initially held by these shareholders was placed with new specialized life sciences investors based in the USA and Europe. The USA represented more than 35% of the placement.

### Net loss reduced and operating expenses stable notwithstanding increased activity level

The net loss for the first half of 2014 amounted to €3.2 million, compared to € 4.1 million for the same period the year before. This decrease of €0.9 million is essentially the result of a reduction of financial charges related to loans that have been converted at the time of the IPO in May 2013, by €1.1 million. This decrease was, partly compensated by somewhat lower grants income and higher operational charges (each by approx. €0.1 million).

The slight increase in operational expenses is caused by a decrease in total Research & Development (R&D) and Clinical trial costs by €0.4 million and an increase in General & Administration (G&A) costs with €0.5 million.

- The decrease in R&D costs (by €0.2 million) is related to reduced development activity on GRASPA® and focalization on the TEDAC and ERY-MET programs;
- Lower clinical trial costs (also by approximately €0.2 million) are related to the completion of the Phase III ALL study enrollment and to the fact that the costs of the largest trial, the AML Phase II study, are carried by our partner Orphan Europe (Recordati Group);
- The increase in G&A costs are mainly related to the development of our activities in the USA and the increased communication and investor relations activities following our Initial Public Offering (IPO) in May 2013.

### Solid cash balance of € 12.3 million<sup>3</sup>

ERYTECH has a strong balance sheet with cash and cash equivalents of €12.3 million at end of June 2014, compared with €15.1 million on December 31, 2013.

Total cash consumption for the period has been €2.8 million, approx. €0.5 million per month.

ERYTECH's key financial figures for the first half of 2014 compared with the same period the previous year are summarized below:

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

	1H 2014	1H 2013
Sales	0	0
Other income (grants)	722	858
<b>Operating income</b>	<b>722</b>	<b>858</b>
Research & Development	-941	-1,157
Clinical trials	-767	-992
Intellectual property	-207	-198
General & Admin	-1991	-1,450
<b>Total operating costs</b>	<b>-3,905</b>	<b>-3,797</b>
<b>Operating result</b>	<b>-3,183</b>	<b>-2,939</b>
Financial result	4	-1,123
Taxes	-4	6
<b>Net result</b>	<b>-3,184</b>	<b>-4,056</b>

<sup>3</sup> Excluding € 0.2 million cash available in the liquidity plan

The financial report for the semester ending June 30, 2014, approved by the Board of Directors on August 29, 2014, 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 2014: Tuesday, 4 November 2014 (after market close)

### Upcoming participations at investor conferences:

- SG Société Générale Healthcare & Biotechnology Conference, September 24, Paris
- CF&B Large & Midcap Event, October 2-3 in Paris
- BioEurope, November 3-5 in Frankfurt
- Jefferies Global Healthcare Conference, November 19-20 in London
- Salon Actionaria, November 21-22 in Paris
- German Equity Forum, November 25-26 in Frankfurt

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

ERYTECH is a French biopharmaceutical company providing new prospects for cancer patients, particularly those with acute leukemia and selected solid tumors. The company is also developing other indications in solid tumors and certain orphan indications outside oncology.

*ERYTECH is listed on 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).*

*For more information about the Company, please read [About ERYTECH and ERY-ASP/GRASPA](#)*

### Forward-looking information

This document may contain forward-looking statements, forecasts and estimates ("Statements") with respect to the financial situation, the results of operations, the strategy, the project and to the anticipated future performance of ERYTECH Pharma. 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 for which ERYTECH Pharma makes no representations or warranty as to their accuracy or fairness. Furthermore, such Statements only speak as of the date of the publication of this document. ERYTECH disclaims any obligation to update any such Statements except to the extent required by French law.

*For more information about Forward-looking information, please read [Forward-looking information](#)*

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