

## **ERYTECH reported full GRASPA® Phase III results in ALL and provided update on AML Phase IIb at ASCO**

**Lyon (France), June 1, 2015** – ERYTECH Pharma (Euronext Paris - ERYP; OTC US - EYRY), the French biopharmaceutical company that develops innovative ‘tumor starvation’ treatments for acute leukemia and other oncology indications with unmet medical needs, reported complete Phase III results of its pivotal program with GRASPA® in Acute Lymphoblastic Leukemia (ALL) and presented the design of the ongoing Phase IIb study in Acute Myeloid Leukemia (AML) at the 51<sup>st</sup> Annual Meeting of the American Society of Clinical Oncology (ASCO). During an investigator meeting ERYTECH also presented the progress and plans on other development programs.

### **ALL Phase III results**

On Saturday, May 30, Prof. Dr. Yves Bertrand, oncologist at the Institute for Pediatric Hematology and Oncology in Lyon, France, presented full Phase III results of the GRASPIVOTALL trial in a plenary session in a packed Arie Crown theatre (4250 seats).

The title of his presentation was:

*Clinical activity of ERY001 (erythrocyte encapsulated L-asparaginase) and native L-asparaginase (L-ASP) in combination with COOPRALL regimen in Phase III randomized trial in patients with relapsed acute lymphoblastic leukemia (ALL)*

The GRASPIVOTALL is a controlled, randomized, multicenter Phase II/III trial comparing GRASPA® (development name: ERY001) to native L-asparaginase (L-ASP) in children and adults suffering from relapsing or refractory ALL. Positive top line data were made available end of last year and demonstrated that the trial met both of its primary endpoints.

The main conclusions of the study, as presented by Prof. Bertrand, are:

- GRASPA in combination with chemotherapy demonstrated sustained asparaginase activity, which was superior compared to L-ASP, for the treatment of patients with ALL. Duration of asparaginase activity above 100 IU/l was 20.5 days in the GRASPA group versus 9.4 days in the L-ASP control arm (p<0.001).
- GRASPA demonstrated a significantly lower risk of hypersensitivity reactions, compared to L-ASP. No hypersensitivity reactions of any grade were observed in the GRASPA treatment arm, versus 46% in the L-ASP control arm (p<0.001).
- The prolonged asparaginase activity was associated with improvement in Complete Remission (CR) rate. 65% of patients in the GRASPA arm were in CR after the induction phase versus 39% in the control arm (p=0.026).
- Treatment was generally well tolerated, with a lower risk of key events, such as coagulation disorders (35% versus 82%<sup>1</sup>), pancreatic toxicities (27% versus 50%<sup>1</sup>) and hepatic toxicities (19% versus 43%<sup>1</sup>).
- The favorable efficacy and safety profile of GRASPA offers an effective alternative options for patients who have received prior asparaginase therapy, including patients who had experienced prior hypersensitivities to *E.Coli* derived asparaginases.

<sup>1</sup> Percentage of patients with at least one drug-related event during the induction phase

- The plenary session was nicely closed by the designated discussant concluding that he viewed GRASPA as “This is an advance”. The role of the discussant among others is to provide to the oncology community a constructive criticism about the researches, questions addresses, results presented or how the papers reviewed may open up new perspectives in the field.

The presentation will be available on the ASCO website (<http://am.asco.org>).

### **AML Phase IIb update**

On Sunday, May 31, Dr. Xavier Thomas, hemato-oncologist at Hospital Lyon South, presented a poster on the design of the ongoing Phase IIb study in Acute Myeloid Leukemia entitled:

*GRASPA-AML 2012-01 study: A multicenter, open, randomized Phase 2b trial evaluating ERY001 (L-asparaginase encapsulated in red blood cells) plus low-dose cytarabine vs low-dose cytarabine alone, in treatment with newly diagnosed acute myeloid leukemia (AML) elderly patients, unfit for intensive chemotherapy*

The poster has been made available in the [company's website](#).

The GRASPA-AML study was launched mid 2013. Today, close to three quarters of the 123 patients to be enrolled in the study have been treated. Two DSMB reviews, one on 30 patients and one on 60 patients, have been performed. A third DSMB review with a futility analysis was originally planned when 60 patients would have experienced an event (progression of the disease or death). The 60 events have recently been reached, later than expected. Given the very advanced stage of patient recruitment in the study, and in order to save statistical power for the final analysis of the study (limit ‘ $\alpha$ -spending’) it was decided not to perform the futility analysis. Futility analyses are typically performed early in a study to avoid unnecessary burden to patients and costs to the sponsor. A safety data review continues to be foreseen and results are expected towards the end of Q3 2015. Full enrolment in the study is expected by the end of the year.

### **Update on other programs**

During investigator meeting organized by ERYTECH on May 31, the company and five distinguished Key Opinion Leaders provided an update on the use of asparaginase products and on the ongoing programs with GRASPA, both in hemato-oncology and solid tumors.

Dr. Ching-Hon Pui, MD, St. Jude Children Hospital, Memphis, presented an overview of the experience with the use of asparaginases in pediatric ALL. He highlighted the contribution asparaginase has made in improving the prognosis for children affected by ALL, but he also pointed at the need for safer formulations to be able to target the more fragile patient populations, such as children in relapse and high risk patients.

Prof. Dr. Larson, MD, PhD, University of Chicago, continued by presenting how the asparaginase was introduced into adult protocols based on pediatric inspired regimens. He continued by describing how important the completion of the planned dose is (25 weeks and more) and how sustained asparaginase activity effect correlated with of survival outcome, compared to the patients who did not completed their treatment, for multiple reasons, including asparaginase related toxicities.

Prof. Dr. Yves Bertrand, MD, PhD, IHOP Lyon, presented the highlights of the clinical evidence with GRASPA in ALL obtained so far, including the Phase III results he communicated the day before in an oral presentation at the ASCO conference

Dr. Phil Lorenzi, PhD, MD Anderson Cancer Center, Houston, USA, presented a summary of most recent work done around L-asparaginase and supporting its utility exploring in solid tumors as well as using ASNS as a predictive biomarker.

Prof Dr. Pascal Hammel, oncologist at Hospital Bichat-Beaujon in Paris continued with a presentation of the ongoing Phase II study with ERY-ASP<sup>2</sup> in pancreatic carcinoma.

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<sup>2</sup> ERY-ASP is the codename for GRASPA outside the field of acute leukemia and outside Europe. The GRASPA® brandname has been licensed for use in acute leukemia in Europe to Orphan Europe (Recordati), ERYTECH’s European commercial partner in Europe.

ERYTECH Pharma subsequently gave an overview of the other current development programs with GRASPA, notably the Phase IIb study in AML and the preparations of a Phase II study in NH lymphoma and a Phase I study with ERY-MET (methioninase in red blood cells).

## About ASCO

ASCO, the American Society of Clinical Oncology, is a professional oncology society committed to conquering cancer through research, education, prevention and delivery of high-quality patient care. ASCO's Annual Meeting is the world largest event on clinical oncology which annually gathers more than 30,000 specialists interested in the latest achievements in the field of cancer treatment.

## About ERYTECH and ERY-ASP/GRASPA®: [www.erytech.com](http://www.erytech.com)

Founded 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. By encapsulating the asparaginase enzyme in red blood cells, ERYTECH has developed ERY-ASP/GRASPA®, an original treatment that targets cancer cells through "tumor starvation" while significantly reducing the side effects for patients. ERY-ASP/GRASPA® has recently announced positive Phase III data in Acute Lymphoblastic Leukemia (ALL) and is in Phase IIb clinical trial in Acute Myeloid Leukemia (AML) in Europe. The product is also in Phase I/II clinical development in ALL in the USA.

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, current forms of asparaginase cannot be used due to their toxicity. With a presumed improved safety profile, ERY-ASP/GRASPA® is being developed to allow all leukemia patients to be treated, even the most fragile ones, representing a market opportunity of more than EUR 1 billion.

The company is also developing other indications in solid tumors and certain orphan indications outside oncology. A Phase II study in pancreas cancer is ongoing and the company is exploring other solid tumor indications for ERY-ASP.

ERYTECH has obtained orphan drug designations for ERY-ASP/GRASPA® in ALL, AML and pancreas cancer, both in Europe and the USA, and has its own GMP-approved and operational manufacturing site in Lyon (France), and a site for clinical production in Philadelphia (USA). The company has concluded licensing and distribution partnership agreements for ALL and AML in Europe with Orphan Europe (Recordati Group), and for ALL with TEVA in Israel.

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 US under an ADR level 1 program (OTC, ticker EYRY).

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