



## ERYTECH Requested a Pre-BLA Meeting with the FDA to Discuss Path to Approval in ALL

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- **ERYTECH invited by FDA to request a pre-BLA meeting**
- **First step in the marketing approval process with the FDA for eryaspase for the treatment of hypersensitive ALL patients based on the positive results of the NOPHO-sponsored Phase 2 clinical trial**

Lyon (France) and Cambridge, MA (U.S.), April 20, 2021 – ERYTECH Pharma (Nasdaq & Euronext: ERYP), a clinical-stage biopharmaceutical company developing innovative therapies by encapsulating therapeutic drug substances inside red blood cells, today announced the initiation of the process of seeking marketing approval from the U.S. Food and Drug Administration (US FDA) for its lead product candidate eryaspase in patients with acute lymphoblastic leukemia (ALL) who developed hypersensitivity reactions to PEG-asparaginase based on the positive results of the NOPHO-sponsored Phase 2 clinical trial.

Maintaining adequate asparaginase treatment following hypersensitivity to PEG-asparaginase is an important goal when treating patients with ALL. A global shortage of supply *Erwinia*-derived asparaginase, which is the current alternative treatment option to PEG-asparaginase, highlights the need for alternative treatment options for these patients.

Results from the NOPHO-sponsored Phase 2 clinical trial demonstrated that eryaspase in combination with chemotherapy, administered every two weeks, provides a sustained asparaginase enzyme activity level, and is generally well tolerated with few hypersensitivity reactions. The trial results were presented at the 62<sup>nd</sup> ASH Annual Meeting held in December 2020. The investigators positioned eryaspase as a potential attractive treatment option for patients who developed hypersensitivities to PEG-asparaginase.

ERYTECH initiated a dialogue with the US FDA in 2020 to evaluate the potential of eryaspase to be approved for the treatment of hypersensitive ALL patients based on the NOPHO-sponsored Phase 2 clinical trial. The FDA confirmed the unmet medical need and, following the review of a comprehensive data package submitted by ERYTECH, invited the Company to request a pre-BLA meeting to discuss the potential for the NOPHO-sponsored Phase 2 clinical trial to support marketing approval in the United States.

ERYTECH has requested a pre-BLA meeting and subject to the feedback received in that meeting, the Company plans to submit a Biologics License Application (BLA) in the second half of 2021 for its lead product candidate eryaspase for the treatment of hypersensitive ALL patients.

#### About the NOPHO trial (NOR-GRASPALL-2016)

The NOPHO-sponsored Phase 2 clinical trial (NOR-GRASPALL-2016) evaluated the safety and pharmacological profile of eryaspase in ALL patients who had previously experienced hypersensitivity reactions to pegylated asparaginase therapy. The trial was conducted by the Nordic Society of Pediatric Hematology and Oncology (NOPHO) at 21 clinical sites in the Nordic and Baltic countries of Europe and enrolled 55 patients. Primary objectives of the trial were asparaginase enzyme activity and safety. Both endpoints were met.

In the trial, eryaspase demonstrated sustained asparaginase enzyme activity above the threshold of >100 U/L at trough levels, 14 days after first infusion in 54 of the 55 patients treated. 96% of the patients were able to complete the intended course of treatment with asparaginase.

Eryaspase was observed to generally be well tolerated when added to chemotherapy and almost all patients were able to receive the intended courses of asparaginase (median of 5 doses per patient). Of the 55 patients, only 2 patients had severe allergic reaction and withdrew from treatment with eryaspase.

#### About Acute Lymphoblastic Leukemia

Acute lymphoblastic leukemia (ALL) is a cancer of the blood and bone marrow that is the most common type of cancer in children in the US and Europe. More than 13,000 cases are diagnosed in the US and Europe each year with the majority of patients diagnosed before age 20. Asparaginase has been an integral component of ALL treatment for several years but is associated with treatment-limiting hypersensitivity in up to 30% of patients. Discontinuation of asparaginase therapy in ALL patients has been associated with inferior event free survival highlighting the need for additional asparaginase based treatment options.

#### About ERYTECH and eryaspase

ERYTECH is a clinical-stage biopharmaceutical company developing innovative red blood cell-based therapeutics for severe forms of cancer and orphan diseases. Leveraging its proprietary ERYCAPS® platform, which uses a novel technology to encapsulate drug substances inside red blood cells, ERYTECH is developing a pipeline of product candidates for patients with high unmet medical needs. ERYTECH's primary focus is on the development of product candidates that target the altered metabolism of cancer cells by depriving them of amino acids necessary for their growth and survival.

The Company's lead product candidate, eryaspase, which consists of L-asparaginase encapsulated inside donor-derived red blood cells, targets the cancer cell's altered asparagine and glutamine metabolism. Eryaspase is in Phase 3 clinical development for the treatment of second-line pancreatic cancer and in Phase 2 for the treatment of first-line triple-negative breast cancer. An investigator-sponsored Phase 2 clinical trial in patients with acute lymphoblastic leukemia who developed hypersensitivity reactions to PEG-asparaginase was recently completed in the Nordic countries of Europe. Eryaspase is not currently approved in any country.

ERYTECH produces its product candidates for treatment of patients in Europe at its GMP-approved manufacturing site in Lyon, France, and for patients in the United States at its GMP manufacturing site in Princeton, New Jersey, USA.

ERYTECH is listed on the Nasdaq Global Select Market in the United States (ticker: ERYP) and on the Euronext regulated market in Paris (ISIN code:

FR0011471135, ticker: ERYP). ERYTECH is part of the CAC Healthcare, CAC Pharma & Bio, CAC Mid & Small, CAC All Tradable, EnterNext PEA-PME 150 and Next Biotech indexes.

For more information, please visit [www.erytech.com](http://www.erytech.com)

## Forward-looking information

This press release contains forward-looking statements including, but not limited to, statements with respect to the clinical development and regulatory plans of eryaspase including the Company's anticipated timing of filing a BLA, the Company's ability to obtain regulatory approval for the treatment of patients with acute lymphoblastic leukemia who developed hypersensitivity reactions to PEG-asparaginase, and the Company's expectations regarding alternative markets for hypersensitive ALL patients. 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. 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 and timeline may turn out to be materially different from the anticipated future results, performance or achievements expressed or implied by such statements, forecasts and estimates. Further description of these risks, uncertainties and other risks can be found in the Company's regulatory filings with the French Autorité des Marchés Financiers (AMF), the Company's Securities and Exchange Commission (SEC) filings and reports, including in the Company's 2020 Document d'Enregistrement Universel filed with the AMF on March 8, 2021 and in the Company's Annual Report on Form 20-F filed with the SEC on March 8, 2021 and future filings and reports by the Company. 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. In addition, the COVID-19 pandemic and the associated containment efforts have had a serious adverse impact on the economy, the severity and duration of which are uncertain. Government stabilization efforts will only partially mitigate the consequences. The extent and duration of the impact on the Company's business and operations is highly uncertain, and that impact includes effects on its clinical trial operations and supply chain. Factors that will influence the impact on the Company's business and operations include the duration and extent of the pandemic, the extent of imposed or recommended containment and mitigation measures, and the general economic consequences of the pandemic. The pandemic could have a material adverse impact on the Company's business, operations and financial results for an extended period of time.

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## Attachment

- [ERYTECH PR\\_pre-BLA\\_ALL - FINAL 2](#)