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PHARMAESSENTIA INITIATES PIVOTAL TRIAL OF ROPEGINTERFERON ALFA-2B TO TREAT ESSENTIAL THROMBOCYTHEMIA

With a diversifying pipeline, the company is evaluating applications for pegylated interferon to address underserved hematologic cancers

January 7, 2021, Burlington, MA – PharmaEssentia Corporation (TPEx: 6446), a global biopharmaceutical innovator leveraging deep expertise and proven scientific principles to deliver new biologics in hematology and oncology, today announced the initiation of SURPASS ET, a Phase 3 pivotal clinical trial of its investigational ropeginterferon alfa-2b (P1101), a novel mono-pegylated proline interferon under evaluation for the treatment of the essential thrombocythemia (ET), one form of myeloproliferative neoplasms (MPNs).

MPNs are caused by specific genetic mutations that lead to overproduction of blood components, including white or red blood cells. ET is one of the group of MPNs, caused by an overproduction of platelets. The disease, which is estimated to affect up to 57 per 100,000 people in the U.S, initially presents with symptoms such as fatigue, anemia and splenomegaly. Over time, ET is known to evolve into myelofibrotic phases with increasingly debilitating symptoms and greater mortality.[1]

“Through our advanced technology, we are working to introduce a new perspective for treating hematologic malignancies such as ET, which need new therapies with the potential to modify and better control the disease,” said Dr. Albert Qin, Chief Medical Officer of PharmaEssentia. “Our goal with this important study is to determine if ropeginterferon alfa-2b may represent a potential solution that can help physicians significantly improve the therapy outcomes for patients in need.”

The multi-site, open-label, randomized, active-controlled study will compare the efficacy, safety, and tolerability and pharmacokinetics of P1101 as second line therapy for ET as compared with anagrelide, a commonly used oral platelet-reduction therapy. The study aims to enroll approximately 160 patients, who will be randomized to receive either P1101 subcutaneously every two weeks or anagrelide at a standard dose. Eligible patients will include high-risk ET patients, those who are resistant to or intolerant of hydroxyurea and who have not received prior therapy with interferon.

The primary endpoint for the study is patient response, as defined by blood count remission, improvement or non-progression in disease related signs, improvement or non-progression in large symptoms and absence of thrombotic events. Topline data are expected by late 2021. More details on the study design and sites are available at www.clinicaltrials.gov (NCT04285086) or at www.SURPASSET.com.

The study builds on the company’s recent momentum to deliver new solutions in MPNs. The company filed a Biological License Application (BLA) with the U.S. Food and Drug Administration (FDA) for Ropeginterferon alfa-2b. The company is seeking approval for its first product indication in polycythemia vera (PV) in the US, and expects an agency decision early in 2021.

About Ropeginterferon alfa-2b

Ropeginterferon alfa-2b is a long-acting, mono-pegylated proline interferon aimed to be administered once every two weeks or longer. Ropeginterferon alfa-2b has Orphan Drug designation for treatment of polycythemia vera (PV) in the United States. Marketed as Besremi® in Europe, the product was approved by the European Medicines Agency (EMA) in 2019. Ropeginterferon alfa-2b was discovered and is manufactured by PharmaEssentia in its Taichung plant, which was cGMP certified by TFDA in 2017 and by EMA in January 2018.

About Essential Thrombocythemia

Essential thrombocythemia (ET) is a myeloproliferative neoplasm (MPN) characterized by an overproduction of platelets in the blood that results from a genetic mutation; data indicates a JAK2 gene mutation is present in approximately half of diagnosed patients. The disease is most commonly diagnosed through routine blood work, and is most common in people over the age of 50, with women 1.5 more times more likely to be diagnosed than men. As a chronic, progressive disease, ET requires regular monitoring and appropriate treatment. Over time, the disease may progress into more deadly conditions such as myelofibrosis or acute leukemia.[1,2]

About PharmaEssentia

PharmaEssentia Corporation (TPEx: 6446) is a rapidly growing biopharmaceutical innovator. Leveraging deep expertise and proven scientific principles, the company aims to deliver effective new biologics for challenging diseases in the areas of hematology and oncology, with one product already approved in Europe and a diversifying pipeline. Founded in 2003 by a team of Taiwanese-American executives and renowned scientists from U.S. biotechnology and pharmaceutical companies, today the company is expanding its global presence with operations in the U.S., Japan, China, and Korea, along with a world-class biologics production facility in Taichung.

Forward Looking Statement

Some of the statements included in this press release, particularly those relating to the results of clinical trials, the clinical benefits to be derived from ropeginterferon alfa-2b, regulatory submissions and the timing of any such review, approvals, the commercial opportunity and competitive positioning, and any business prospects for ropeginterferon alfa-2b, may be forward-looking statements that involve a number of risks and uncertainties. For those statements, we claim the protection of the safe harbor for forward-looking statements contained in the Private Securities Litigation Reform Act of 1995 and similar legislation and regulations under Taiwanese law. Among the factors that could cause our actual results to differ materially

are the following: acceptance of the BLA filing does not represent final evaluation of the adequacy of the data submitted in the BLA; whether the FDA will complete its review of the BLA on a timely basis; the risk that the FDA ultimately denies approval of the BLA; whether the FDA concurs with our interpretation of our Phase 3 study results, supportive data, or the conduct of the studies; whether, ropeginterferon alfa-2b, if approved, will be successfully launched and marketed; and other risk factors identified from time to time in our reports filed with any global securities regulator or agency. Any forward-looking statements set forth in this press release speak only as of the date of this press release. We do not undertake to update any of these forward-looking statements to reflect events or circumstances that occur after the date hereof. The information found on our website, and the FDA website, is not incorporated by reference into this press release and is included for reference purposes only.

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[1] Mehta J, Wang H, Iqbal SU, Mesa R. Epidemiology of myeloproliferative neoplasms in the United States. Leuk Lymphoma. 2014 Mar;55(3):595-600

[2] "What is Essential Thrombocythemia?" MPN Research Foundation. 2020. Available at: http://www.mpnresearchfoundation.org/Essential-Thrombocythemia