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# PHARMAESSENTIA INITIATES PHASE 2B TRIAL OF ROPEGINTERFERON ALFA-2B-NJFT FOR ESSENTIAL THROMBOCYTHEMIA (ET) IN NORTH AMERICA

*Single-arm trial to expand body of evidence supporting treatment with novel monopegylated interferon among adults with ET living in the U.S. and Canada*

February 23, 2023, Burlington, MA – PharmaEssentia USA Corporation, a subsidiary of PharmaEssentia Corporation (TPEx:6446), a global biopharmaceutical innovator based in Taiwan leveraging deep expertise and proven scientific principles to deliver new biologics in hematology and oncology, today announced that the first patients are now being dosed in a Phase 2b clinical study evaluating ropeginterferon alfa-2b-njft for the treatment of adults with essential thrombocythemia (ET).

ET is a rare blood cancer characterized by a genetic mutation triggering the overproduction of platelets in the blood. It is part of a group of disorders called myeloproliferative neoplasms (MPNs), for which there are few therapeutic options today. Without proper treatment, ET often progresses toward myelofibrosis or secondary acute myeloid leukemia (sAML). Based on an improved understanding of the disease etiology, clinical guidelines recommend the use of systemic therapies that can move beyond symptom management toward more complete control of the disease.

“Clinicians who treat ET patients recognize the need for more effective and more tolerable treatment options to gain greater control over the effects of this progressive cancer and help more patients reach their goals,” said Lucia Masarova, M.D., Assistant Professor, Department of Leukemia at UT MD Anderson Cancer Center, Houston Texas. “This important study will provide useful insights into the role of this novel interferon to overcome the limitations of the current options available today and potentially represent a real advancement in the care of these patients.”

This single-arm study will assess the efficacy of ropeginterferon alfa-2b-njft in approximately 64 eligible adults with ET at 20-25 treatment centers in the United States and Canada. The study will include assessments of hematologic response rate as well as changes in the JAK2 and CALR mutation to understand their role in treatment response, and will capture additional safety and tolerability assessments, quality of life impact, and other evaluations. The study will include a 12-month primary treatment phase during which participants will receive treatment subcutaneously every two weeks (starting dose 250 mcg, target optimal dose 500 mcg) followed by a 28-day follow up. Those who respond to treatment will be eligible to participate in an extension phase of the study. More information on the study including eligibility criteria can be found by visiting www.ExceedET.com or www.clinicaltrials.gov and searching for the trial identifier NCT05482971.

“Years of ongoing research have demonstrated that there is an important role for a monopegylated interferon to improve the care paradigm for people who suffer from chronic, progressive myeloproliferative neoplasms. We’ve delivered on this through our first indication in polycythemia vera, and now we are expanding our focus into ET,” said Raymond Urbanski, M.D., Ph.D., U.S. Head of Clinical Development and Medical Affairs. “Our goal through this research is

to provide a clear, comprehensive view of the clinical profile for this treatment in ET that could support registration."

This study will build upon the insights generated as part of the ongoing global SURPASS ET study, a Phase 3 pivotal clinical trial of ropeginterferon alfa-2b for the treatment of ET. Topline data for both trials are expected in 2024.

**About BESREMi® (ropeginterferon alfa-2b-njft)**

BESREMi is an innovative monopegylated, long-acting interferon. With its unique pegylation technology, BESREMi has a long duration of activity in the body and is aimed to be administered once every two weeks (or every four weeks with hematological stability for at least one year), allowing flexible dosing that helps meet the individual needs of patients.

BESREMi has orphan drug designation for the treatment of polycythemia vera (PV) in adults in the United States. The product was approved by the European Medicines Agency (EMA) in 2019, in the United States in 2021, and has recently received approval in Taiwan and South Korea. The drug candidate was invented by PharmaEssentia and is manufactured in the company's Taichung plant, which was cGMP certified by TFDA in 2017 and by EMA in January 2018. PharmaEssentia retains full global intellectual property rights for the product in all indications.

BESREMi was approved with a boxed warning for risk of serious disorders including aggravation of neuropsychiatric, autoimmune, ischemic and infectious disorders.

Please see full Prescribing Information, including Boxed Warning.

**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. ET is estimated to affect up to 57 per 100,000 people in the U.S. 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 (TPEx: 6446), headquartered in Taipei, Taiwan, is a global and rapidly growing biopharmaceutical innovator. Leveraging deep expertise and proven scientific principles, PharmaEssentia aims to deliver effective new biologics for challenging diseases in the areas of hematology and oncology, with one approved product 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 PharmaEssentia 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, Taiwan.

For more information, visit our website or find us on LinkedIn and Twitter.

**Forward Looking Statement**

This press release may contain forward-looking statements. 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. These forward-looking statements are based on management expectations and assumptions as of the date of this press release, and actual results may differ materially from those in these forward-looking statements as a result of various factors. These factors include risks and uncertainties related to the initiation, timing, progress and results of our research and development programs, preclinical studies, clinical trials, and regulatory submissions. We do not undertake to update any of these forward-looking statements to reflect events or circumstances that occur after the date hereof.

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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