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FIVE-YEAR DATA ON ROPEGINTERFERON ALFA-2B ILLUSTRATE DURABLE RESPONSE AT FIVE YEARS IN PEOPLE WITH POLYCYTHEMIA VERA

Presentation at Virtual ASH Annual Meeting found treatment with ropeginterferon alfa-2b at five years reduced drivers of disease progression, including allelic burden

December 7, 2020, 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 long-term results with ropeginterferon alfa-2b, an investigational product, which showed patients with polycythemia vera (PV) experienced durable rates of hematologic and molecular responses after five years of treatment.[1] These results were featured as an oral presentation at the virtual 62nd American Society of Hematology (ASH) Annual Meeting and Exposition and presented by author Heinz Gisslinger, M.D. of Medical University Vienna in Austria. Ropeginterferon alfa-2b (INN, PharmaEssentia) was invented and is manufactured by PharmaEssentia. This clinical study was conducted and sponsored by AOP Orphan Pharmaceuticals.

PV is a rare, chronic blood cancer that occurs when the bone marrow produces excessive red blood cells, causing the blood to be thicker than normal and potentially leading to a range of complications.[2,3] Most cases are caused by a JAK2 V617F mutation[2,3] and, without proper management, PV can progress into myelofibrosis and malignancies, including acute myeloid leukemia.[4]

“As a clinician treating a chronic blood cancer, it’s valuable to see data on how ropeginterferon alfa-2b can help more patients achieve complete hematologic control of their polycythemia vera over time,” said Jean-Jacques Kiladjian, M.D., Ph.D. of Saint-Louis Hospital and Paris Diderot University in France and study author. “It’s particularly encouraging to observe the sustained molecular response in this five-year data, as this will be a critical measure to help us manage disease progression in this population.”

This five-year data from the PROUD-PV/CONTINUATION-PV studies demonstrates the short- and long-term effects of ropeginterferon alfa-2b among PV patients. The majority of patients (81.8%) maintained hematocrit levels less than 45 percent without the need for phlebotomy and had low rates of thromboembolic events (4.2%). With long-term treatment, patients also experienced significantly reduced median allele burden (7.3% vs. 37.3% at baseline).

More than two-thirds of patients (69.1%) who received ropeginterferon alfa-2b experienced a molecular response, which was accompanied by a low risk of disease progression. During the entire study period, there was one reported case of progression to myelofibrosis and no cases of leukemic transformation among those treated with ropeginterferon alfa-2b. Importantly, more than half of the participants in the study (58.5%) achieved complete response, including both a well-controlled hematocrit without needing phlebotomy as well as a molecular response – parameters known to influence the risk of progression in PV. No new safety or tolerability signals were identified in the study period. Over the entire treatment period, treatment-related adverse events and discontinuation rates were similar among the treatment groups. The rate of grade ≥3 drug-related adverse events in each study arm was 16.5 percent.[1]

"These are the first and longest phase 3 studies to evaluate an interferon in polycythemia vera, a disease where there are significant unmet needs to help more patients control the disease and help to reduce the risk of progression to more deadly malignancies," said Raymond Urbanski, M.D., Ph.D., Head of U.S. Clinical Development and Medical Affairs for PharmaEssentia. "These results reinforce and strengthen previously reported findings on this novel long-acting interferon and its potential to offer benefit to the community."

PharmaEssentia submitted a Biologics License Application to the U.S. Food and Drug Administration (FDA) for review of ropeginterferon alfa-2b earlier this year and anticipates an agency decision in early 2021.

About the Ropeginterferon Alfa-2b Clinical Trials

PROUD-PV is a randomized, controlled, multicenter phase 3 study that evaluated the efficacy, safety and tolerability of ropeginterferon alfa-2b compared to hydroxyurea in 257 adult patients with polycythemia vera (PV) new to cytoreductive treatment or treated with hydroxyurea for less than three years. The study met the primary endpoint of noninferior complete hematological response rate at 12 months and secondary outcomes included hematologic response rate at three, six and nine months, mutant JAK2 allelic burden changes, and molecular response rate, among others. The study was conducted by AOP Orphan Pharmaceuticals in Europe.

CONTINUATION-PV is an open-label, multicenter, phase 3b extension of the PROUD-PV study assessing the long-term safety and efficacy of ropeginterferon alfa-2b compared to best available therapy in 171 patients with PV. Efficacy assessments included hematologic parameters, phlebotomy need, JAK2V617F allele burden, and molecular response defined by modified ELN criteria. An interim analysis was performed once all patients were treated for five years. Results from the studies were published in *The Lancet Haematology* earlier this year.

About Ropeginterferon alfa-2b

Ropeginterferon alfa-2b is a novel, 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. The novel ropeginterferon alfa-2b was invented and is manufactured by PharmaEssentia in its Taichung plant, which was cGMP certified by TFDA in 2017 and by the EMA in January 2018.

About Polycythemia Vera

Polycythemia Vera (PV) is a cancer originating from a disease-initiating stem cell in the bone marrow resulting in a chronic increase of red blood cells, white blood cells, and platelets. This condition may result in cardiovascular complications such as thrombosis and embolism, as well as transformation to secondary myelofibrosis or leukemia. While the molecular mechanism underlying PV is still subject of intense research, current results point to a set of acquired mutations, the most important being a mutant form of JAK2.[4]

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

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] Gisslinger, H, Klade, C, et. al. Long-Term Use of Ropeginterferon Alpha-2b in Polycythemia Vera: 5-Year Results from a Randomized Controlled Study and Its Extension. Presentation #481 in Session #634. Presented virtually at the 62nd American Society of Hematology (ASH) Annual Meeting and Exposition; December 5-8.
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[3] Mesa R, et al. Patient-Reported Outcomes Data from REVEAL at the Time of Enrollment (Baseline): A Prospective Observational Study of Patients With Polycythemia Vera in the United States. Clin Lymphoma Myeloma Leuk. 2018 Sep;18(9):590-596. doi: 10.1016/j.clml.2018.05.020.

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