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U.S. FDA ACCEPTS PHARMAESSENTIA'S APPLICATION FOR ROPEGINTERFERON ALFA-2B TO TREAT POLYCYTHEMIA VERA

U.S. team begins commercial preparations for novel pegylated interferon with appointment of General Manager Meredith Manning

June 4, 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 that the U.S. Food and Drug Administration (FDA) has recently accepted its Biologics License Application (BLA) for ropeginterferon alfa-2b (P1101), a novel pegylated interferon intended for the treatment of the rare blood cancer polycythemia vera (PV) in the absence of symptomatic splenomegaly. The company expects an agency decision in early 2021.

PharmaEssentia has focused its efforts on therapeutic innovation in the category of myeloproliferative neoplasms (MPNs), which are caused by specific genetic mutations that lead to overproduction of blood components including white or red blood cells, or platelets. In PV, which is caused by a JAK2 V617F mutation, the bone marrow produces excessive red blood cells, causing the blood to be thicker than normal and potentially leading to a range of complications.[1,2] PV is estimated to affect more than 160,000 people in the U.S. alone,[1] who have progressively burdensome symptoms. Without proper management, the disease progresses into malignancies including myelofibrosis and acute myeloid leukemia.[3]

Ropeginterferon alfa-2b was invented by scientists at PharmaEssentia in Taiwan and is a structurally novel monopegylated proline interferon designed for administration once every two weeks. The U.S. filing is supported by robust, durable 24-36-month data from the Phase 3 PROUD/CONTI-PV clinical trial, which demonstrated that the investigational treatment offered high and durable hematologic responses and symptom control with good tolerability and low rates of depression observed, with effects on relevant MPN mutations supporting a potential disease modifying capability.[4]

The findings were shown among patients who received either Ropeginterferon alfa-2b (n=95) or hydroxyurea/best available therapy (HU/BAT, n=74). At 36 months of treatment, patients who received Ropeginterferon alfa-2b maintained a complete hematological response longer than those who received HU/BAT (70.5% vs. 51.4%). Response rates steadily increased in the Ropeginterferon alfa-2b arm throughout 24 months of treatment and remained constant after 36 months. Further, after 36 months, two thirds (66.0%) of patients who received Ropeginterferon alfa-2b achieved a molecular response, compared with 27% in the HU/BAT arm. Importantly, these molecular responses were closely related to complete hematological responses. There were similar rates of adverse events in both arms; the most common (>10%) treatment-related adverse events included anemia, thrombocytopenia and leukopenia, which occurred more frequently under HU.[4]

"Our focus is on stunting these rare malignancies, preserving patient well-being and slowing the progression into more aggressive and deadly cancers," said Meredith Manning, U.S. General Manager for PharmaEssentia. "We believe Ropeginterferon alfa-2b could become an important

new therapeutic tool and look forward to engaging with the regulators in our efforts to introduce this option to the underserved PV community in the U.S."

Ms. Manning was recently appointed to the U.S. GM role to guide the expansion of the company's U.S. presence, with near-term focus on the commercial preparations for the first target indication in PV. Ms. Manning brings PharmaEssentia dynamic expertise in commercialization and market strategy. She joined from resTORbio, where she served as Chief Commercial Officer to define the corporate strategy and the commercial launch approach for an aging-related therapeutic.

About Ropeginterferon alfa-2b

Ropeginterferon alfa-2b is a novel, long-acting, mono-pegylated proline interferon that has been engineered with an optimized profile to support improved pharmacokinetic properties and demonstrated tolerability and convenience compared with conventional interferons. It is designed for administration once every two weeks, or once every four weeks during long-term maintenance. 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 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.[3]

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.

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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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