Hepcidin Mimetic PTG-300 Receives U.S. FDA Orphan Drug Designation for the Treatment of Polycythemia Vera

June 18, 2020

NEWARK, Calif., June 17, 2020 /PRNewswire/ -- Protagonist Therapeutics, Inc. (Nasdaq: PTGX) today announced that the U.S. Food and Drug Administration (FDA) has granted Orphan Drug Designation for PTG-300 for the treatment of polycythemia vera. PTG-300 is an injectable synthetic peptide mimetic of the natural hormone hepcidin currently in clinical development for the treatment of polycythemia vera and hereditary hemochromatosis.

"Receiving FDA orphan drug designation is another important milestone for Protagonist and underscores the importance of our work in polycythemia vera," commented Samuel Saks, M.D., Protagonist Chief Medical Officer. "Individuals living with polycythemia vera face a high disease burden. PTG-300 has a non-cytoreductive therapeutic mechanism in the treatment of polycythemia vera and has shown a well-tolerated safety profile to date. Because of its properties, PTG-300 may help provide sustained control of hematocrit and potentially help address symptoms of polycythemia vera and systemic iron deficiency in these patients."

Protagonist recently announced initial Phase 2 results in patients with polycythemia vera that demonstrated robust clinical response and clinically meaningful dose related control of hematocrit levels on individual patient basis.

About Polycythemia Vera

Polycythemia vera is a myeloproliferative neoplasm characterized primarily by the increased production of red blood cells. Well-established treatment guidelines focus on maintaining hematocrit levels below 45 percent to reduce the risk of thrombotic events. Unfortunately, current treatment options are unable to maintain hematocrit to below the 45 percent target for many patients. In addition, current options are intolerable to some patients and may be associated with serious side effects, such as exacerbation of iron deficiency with phlebotomy. There are an estimated 100,000 patients with polycythemia vera in the U.S. and approximately 100,000 patients in major EU countries. Patients are classified as either low risk or high risk based on prior thrombotic events and age. A treatment option that could provide consistent control of hematocrit over time without fluctuations above 45 percent could be an important component of care for both low and high risk polycythemia vera patient populations.

About Orphan Drug Designation

The FDA grants Orphan Drug Designation to novel drugs or biologics that treat rare diseases or conditions affecting fewer than 200,000 patients in the U.S. The designation allows the sponsor of the drug to be eligible for a seven-year period of U.S. marketing exclusivity on approval of the drug, as well as tax credits for clinical research costs, the ability to apply for annual grant funding, clinical trial design assistance, and the waiver of Prescription Drug User Fee Act (PDUFA) filing fees.

About Protagonist Therapeutics, Inc.

Protagonist Therapeutics is a clinical stage biopharmaceutical company that utilizes a proprietary technology platform to discover and develop novel peptide-based therapeutics to address significant unmet medical needs and transform existing treatment paradigms for patients. The Company currently has three clinical-stage assets. PTG-300 is an injectable hepcidin mimetic in development for the treatment of polycythemia vera and hereditary hemochromatosis. PTG-200 is an orally delivered, gut-restricted, interleukin-23 receptor specific antagonist peptide in development for the treatment of inflammatory bowel disease, with Crohn's disease as the initial indication. The Company has a worldwide license and collaboration agreement with Janssen Biotech, Inc., for the development of PTG-200. PN-943 is an orally delivered, gut-restricted alpha-4-beta-7 integrin specific antagonist peptide in development for the treatment of inflammatory bowel disease, with ulcerative colitis as the initial targeted indication.

Protagonist is headquartered in Newark, California. For further information, please visit www.protagonist-inc.com.

Cautionary Note on Forward-Looking Statements

This press release contains forward-looking statements for purposes of the safe harbor provisions of the Private Securities Litigation Reform Act of 1995. Forward-looking statements include statements regarding our intentions or current expectations concerning, among other things, the potential for our clinical programs, the potential of PTG-300 as a possible treatment for polycythemia vera and hereditary hemochromatosis, the safety profile of PTG-300, the potential for PTG-300 to provide sustained control of hematocrit in patients, the potential for PTG-300 to help address symptoms of polycythemia vera and systemic iron deficiency in patients, the results of the Phase 2 study of PTG-300 in polycythemia vera and the results of future studies for the treatment of polycythemia vera. In some cases, you can identify these statements by forward-looking words such as "may," "potential," or the negative or plural of these words or similar expressions. Forward-looking statements are not guarantees of future performance and are subject to risks and uncertainties that could cause actual results and events to differ materially from those anticipated, including, but not limited to, our ability to develop and commercialize our product candidates, our ability to earn milestone payments under our collaboration agreement with Janssen, our ability to use and expand our programs to build a pipeline of product candidates, our ability to obtain and maintain regulatory approval of our product candidates and risks related to the global COVID-19 pandemic and actions taken to slow its spread. Additional information concerning these and other risk factors affecting our business can be found in our periodic filings with the Securities and Exchange Commission, including under the heading "Risk Factors" contained in our Quarterly Report on Form 10-Q for the period ended March 31, 2020, filed with the Securities and Exchange Commission. Forward-looking statements are not guarantees of future performance, and our actual results of operations, financial condition and liquidity, and the development of the industry in which we operate may differ materially from the forward-looking statements contained in this press release. Any forward-looking statements that we make in this press release speak only as of the date of this press release. We assume no obligation to update our forward-looking statements, whether as a result of new information, future events or otherwise, after the date of this press release.

View original content:http://www.prnewswire.com/news-releases/hepcidin-mimetic-ptg-300-receives-us-fda-orphan-drug-designation-for-the-treatment-of-polycythemia-vera-301079240.html

SOURCE Protagonist Therapeutics, Inc.

Solebury Trout, Rich Allan (media), Tel: +1 646-378-2958, Email: rallan@soleburytrout.com, Brian Korb (investors), Tel: +1 646-378-2923, Email: bkorb@soleburytrout.com