Protagonist Therapeutics Receives Orphan Drug Designation from the European Medicines Agency for PTG-300 in Polycythemia Vera

October 21, 2020

-- PTG-300 was previously granted orphan drug designation by the U.S. Food and Drug Administration --

NEWARK, Calif., Oct. 21, 2020 /PRNewswire/ -- Protagonist Therapeutics, Inc. (NASDAQ:PTGX) today announced that the European Medicines Agency (EMA) has granted orphan drug designation for PTG-300 in the treatment of polycythemia vera. PTG-300 is an injectable synthetic peptide mimetic of the natural hormone hepcidin and has previously received orphan drug designation for the treatment of polycythemia vera from the U.S. Food and Drug Administration (FDA).

"This designation reflects the potential of PTG-300 as a treatment candidate and the global need for novel treatments for individuals living with polycythemia vera," commented Samuel Saks, M.D., Protagonist Chief Medical Officer. "Early clinical results that were reported in May, as well as the novel non-cytoreductive therapeutic mechanism of PTG-300 in regulating iron, suggest that PTG-300 may help a broad population of polycythemia vera patients. We are currently engaged in discussions with leaders in treatment of polycythemia vera to design a pivotal study. Completion of enrollment for the ongoing study of 50 patients is expected in mid-2021. A pivotal study is expected to begin in the second half of 2021, pending our planned discussions with regulatory agencies."

A Phase 2 study of PTG-300 in patients with polycythemia vera is currently enrolling subjects. Additional information is available at http://ptg300pvstudy.com/.

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 continuously 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 and may be associated with serious side effects. There are an estimated 100,000 patients with polycythemia vera in the U.S. and approximately 100,000 patients in major EU countries.

About the European Medicines Agency (EMA) Orphan Drug Designation

Orphan drug designation in the European Union (EU) is granted by the European Commission based on a positive opinion issued by the EMA Committee for Orphan Medicinal Products. To qualify, a therapeutic candidate must be intended to treat a serious condition that affects fewer than five in 10,000 people in the EU, and there must be sufficient data to suggest the candidate may produce clinically relevant outcomes. The designation provides companies with certain benefits and incentives for clinical development and a period of market exclusivity, if approved.

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 other blood disorders. 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 http://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 benefits of PTG-300 in treating polycythemia vera, the timing of enrollment in our ongoing PTG-300 clinical trial and the commencement of a pivotal study of PTG-300 in polycythemia vera patients. In some cases, you can identify these statements by forward-looking words such as "anticipate," "believe," "may," "will," "expect," 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, the impact of the current COVID-19 pandemic on our discovery and development efforts, 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, our ability to operate in a competitive industry and compete successfully against competitors that have greater resources than we do, and our ability to obtain and adequately protect intellectual property rights for our product candidates. 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 quarter ended June 30, 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.

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