Protagonist Therapeutics Receives FDA Breakthrough Therapy Designation for Rusfertide in Polycythemia Vera

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Designation further validates registrational path for rusfertide in polycythemia vera and facilitates potentially expedited development and review

NEWARK, Calif., June 3, 2021 /PRNewswire/ -- Protagonist Therapeutics ("Protagonist" or "the Company") (Nasdaq: PTGX), today announced that the U.S. Food and Drug Administration (FDA) has granted Breakthrough Therapy Designation for its lead investigational new drug candidate, rusfertide, for the treatment of patients with polycythemia vera (PV) for the reduction of erythrocytosis in those patients who do not require further treatment for thrombocytosis and/or leukocytosis. Breakthrough Therapy Designation requires that the drug candidate treat a serious or life-threatening disease or condition. It also requires preliminary clinical evidence that indicates that the drug may demonstrate substantial improvement over existing therapies on one or more clinically significant endpoints. The designation has the potential to expedite the development and regulatory review process.

"We are thrilled to receive the Breakthrough Therapy Designation for rusfertide in PV, a serious disease where the need for different and better treatment options is clear and pressing," said Suneel Gupta, PhD, Chief Development Officer at Protagonist. "Rusfertide is a natural hormone mimetic and may stand out as the first non-cytoreductive therapeutic drug for PV. We look forward to working closely with FDA regulators to advance and complete all relevant clinical studies, both ongoing and planned, as quickly as possible."

The designation for rusfertide was supported in part by promising data from the ongoing Phase 2 clinical trial in patients with PV, presented at the Annual Meeting of the American Society of Hematology in 2020. The data showed that when treated with rusfertide, a majority of patients were able to eliminate therapeutic phlebotomies, maintain a target hematocrit level of less than 45 percent, reverse iron deficiency, and experience symptom improvements. The FDA previously granted orphan drug status and Fast Track Designation to rusfertide in PV. Breakthrough Therapy Designation offers additional advantages over Fast Track Designation, including FDA actions to expedite both planned clinical trials and plans for expediting the manufacturing development strategy.

Updated data from the ongoing Phase 2 study has been selected for oral presentation at the upcoming annual meeting of the European Hematology Association. This meeting will take place June 9 through 17 and will remain accessible until August 15, 2021.

About FDA Breakthrough Therapy Designation

Breakthrough Therapy Designation is an FDA program intended to expedite the development and regulatory review of investigational therapies that are designed to address serious or life-threatening conditions. The criteria for Breakthrough Therapy Designation requires preliminary clinical evidence that indicates that the candidate may demonstrate substantial improvement over existing therapies on one or more clinically significant endpoints. This designation provides the Company with more intensive FDA guidance on an efficient drug development program, and eligibility for other actions to expedite the FDA review, such as a rolling review of a New Drug Application (NDA), where the FDA may review sections of the NDA before the complete application is submitted. An NDA for a product candidate receiving Breakthrough Therapy Designation may also be eligible for priority review if the relevant criteria are met. Breakthrough Therapy Designation does not change the standards for approval. For more information, please visit the FDA website at www.fda.gov.

About Protagonist Therapeutics

Protagonist Therapeutics is a biopharmaceutical company with multiple peptide-based investigational new chemical entities in different stages of development, all derived from the Company's proprietary technology platform.

Protagonist-developed compounds currently include rusfertide (PTG-300), an investigational, injectable hepcidin mimetic in a Phase 2 proof-of-concept clinical trial for polycythemia vera, and a separate Phase 2 clinical study for hereditary hemochromatosis. Based on the feedback provided by the FDA and EU regulatory authorities, the Company plans to initiate a single, global Phase 3 randomized, placebo-controlled trial evaluating the efficacy and safety of a once weekly, subcutaneously self-administered dose of rusfertide. Additionally, PN-943 is an investigational orally delivered, gut-restricted alpha-4-beta-7 integrin specific antagonist peptide currently in a Phase 2 study for the potential treatment of inflammatory bowel disease, with ulcerative colitis as the initial targeted indication.

The Company has a worldwide license and collaboration agreement with Janssen Biotech, Inc., for the development of oral peptide IL-23 receptor antagonists. Compounds included in this agreement are PTG-200, PN-235 and PN-232. PTG-200 is an orally delivered, gut-restricted, interleukin-23 receptor specific antagonist peptide in a Phase 2 clinical trial for Crohn's disease. PN-235 and PN-232, both second-generation oral interleukin-23 receptor antagonist candidates, are in Phase 1 studies.

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, among other things, our plans to present updated rusfertide data at EHA and conduct a Phase 3 trial evaluating rusfertide for PV. 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, our ability to earn milestone payments under our collaboration agreements, 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 most recently filed periodic reports on Form 10-K and Form 10-Q 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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Investor Relations & Media: Jami Taylor, Protagonist Therapeutics, Tel: +1.510.474.0170 ext. 300, Email: j.taylor@ptgx-inc.com