Protagonist Therapeutics Announces Initial Phase 2 Results with Hepcidin Mimetic PTG-300 in the Treatment of Polycythemia Vera

May 7, 2020

-- PTG-300 treatment demonstrates robust clinical response and provides clinically meaningful dose related control of hematocrit values on individual patient basis --

-- Results to be presented today by Ronald Hoffman, M.D., Director of the Myeloproliferative Diseases Program at The Icahn School of Medicine at Mount Sinai --

NEWARK, Calif., May 7, 2020 /PRNewswire/ -- Protagonist Therapeutics, Inc. (Nasdaq:PTGX) today announced initial data from the ongoing Phase 2 study of PTG-300 in patients with polycythemia vera. The current results demonstrate that treatment with PTG-300 at individualized doses ranging from 10 mg to 80 mg for up to 28 weeks provided dose-related control of hematocrit levels and eliminated the need for phlebotomy in all six out of six patients that received the dosing as per protocol. A seventh patient with 12 weeks of treatment had an unintended dose interruption, received a single phlebotomy, and remains on the study. In addition, positive symptomatic measurements related to the ability of PTG-300 to address iron deficiency in these frequently phlebotomized patients were observed, with increases in serum ferritin values approaching the range observed in healthy subjects. Patients enrolled in the current study had received at least three phlebotomies within a 24 week period prior to PTG-300 treatment and were treated for up to 28 weeks as of the cutoff date of May 1, 2020 (range of 4 to 28 weeks, n=7 evaluable for efficacy). Enrollment in the study continues and a total of eight patients have enrolled to date.

"While further follow up and data from additional patients will be needed to confirm the continuity of the robust clinical responses observed to date, we believe that this study provides a compelling rationale to initiate planning for a pivotal program in polycythemia vera," commented Samuel Saks, M.D., Protagonist Chief Medical Officer. "As a peptide mimetic of the natural hepcidin hormone, PTG-300 is believed to limit the excess number of red blood cells in polycythemia vera by reducing iron available for red blood cell production. In the near term, we are expanding the current study to include additional patients as the Company focuses on these encouraging results. We will also be hosting a scientific planning meeting with leaders in the field of myeloproliferative neoplasms and working with patient advocates to discuss pivotal and future studies in polycythemia vera. Our goal with these studies is to work to address the broad populations of patients that may benefit from this new non-cytoreductive treatment."

"These initial data demonstrate the potential of PTG-300 to almost entirely avoid the need for phlebotomy in the treatment of polycythemia vera by persistent control of hematocrit levels to below 45 percent," commented Ronald Hoffman, M.D., Director of the Myeloproliferative Diseases Program at The Icahn School of Medicine at Mount Sinai and an investigator in the PTG-300 polycythemia vera study. "Previous studies have repetitively demonstrated that patients undergoing phlebotomy in addition to other therapies spend far too much time above the target hematocrit levels of 45 percent in the clinical guidelines. This is despite the fact that hematocrit levels above this target are associated with significant cardiovascular events such as heart attack and stroke. PTG-300 offers the possibility of maintaining patients consistently below 45 percent hematocrit levels with weekly administration of a mimetic of the endogenous iron regulator without the up and down excursions inherent in typical phlebotomy therapy. In addition, the reduction in phlebotomy may allow sufficient iron to be available systemically to avoid symptoms related to iron deficiency. The potential for weekly self-administration with PTG-300 is a meaningful advantage of this approach to treatment. These early results are very encouraging and suggest the potential for a paradigm shift for the treatment of polycythemia vera. We look forward to additional data from the expanded study in the future."

Administration of PTG-300 was well tolerated and the safety profile was generally similar with results of prior studies, with injection site reactions and bruise as the only observed adverse events. With eight subjects enrolled to date, the study continues to accrue patients and none of the patients have discontinued treatment with PTG-300.

The study is designed to monitor the safety profile and to obtain evidence of efficacy in patients requiring frequent phlebotomies. Based on the initial findings, the study is being expanded and is now expected to enroll approximately 50 patients. The study design consists of a 16-week open-label dose escalation, reduction, or maintenance stage every four weeks from 10 mg to 80 mg and a 12-week maintenance period at doses that generate desired hematocrit levels followed by a randomized and blinded withdrawal stage up to 12 weeks. The study has an open-label extension for up to one year to monitor long-term safety and other effects. The primary endpoint is the proportion of responders during the blinded randomized withdrawal period. Other endpoints of this clinical proof-of-concept study include measurement of blood parameters (hematocrit and hemoglobin levels), reductions or delay in phlebotomy requirements and improvements in quality-of-life symptoms. Additional information is available at https://clinicaltrials.gov/ct2/show/NCT04057040.

Conference Call and Webcast Information

Protagonist will host a conference call at 5 p.m. EDT / 2 p.m. PDT today to provide a corporate update. Ronald Hoffman, M.D., Director of the Myeloproliferative Diseases Program at The Icahn School of Medicine at Mount Sinai, will join the call to present initial results for PTG-300 in polycythemia vera. To access the live call, dial 1-844-515-9178 (U.S./Canada) or 1-614-999-9313 (international) and refer to conference ID number 4597494. A live and archived webcast will also be accessible in the Investors section of the Company's website at www.protagonist-inc.com.

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, the Company's success at finding appropriate doses of PTG-300 for the treatment of polycythemia vera, planning for a pivotal program in polycythemia vera, the results of the Phase 2 study of PTG-300 in polycythemia vera, the results of future studies for the treatment of polycythemia vera, plans for future clinical trials, the initiation and availability of results of our clinical trials and the sufficiency of our financial resources, our ability to fund our clinical trials, the initiation of and enrollment of patients in our clinical trials including trails related to PTG-300 as a possible treatment for polycythemia vera, the results of clinical trials and the outlook for our other programs. In some cases, you can identify these statements by forward-looking words such as "will," "plan," "believe," "may," "potential," "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 agreement with Janssen, our ability to use and expand our programs to build a pipeline of product candidates, and our ability to obtain and maintain regulatory approval of 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 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 to download multimedia:http://www.prnewswire.com/news-releases/protagonist-therapeutics-announces-initial-phase-2-results-with-hepcidin-mimetic-ptg-300-in-the-treatment-of-polycythemia-vera-301055194.html

SOURCE Protagonist Therapeutics, Inc.

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