Proteon Therapeutics Announces Publication of Results from Phase 3 PATENCY-1 Clinical Trial of Investigational Vonapanitase in Patients with Chronic Kidney Disease

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Results supported the design of ongoing pivotal study and FDA Breakthrough Therapy designation

WALTHAM, Mass., Jan. 24, 2019 (GLOBE NEWSWIRE) -- Proteon Therapeutics Inc. (Nasdaq: PRTO), a company developing novel, first-in-class pharmaceuticals to address the medical needs of patients with kidney and vascular diseases, today announced the publication in the *Journal of Vascular Surgery* of results from its Phase 3 PATENCY-1 clinical trial of investigational vonapanitase. Top-line data from PATENCY-1 were publicly announced in December 2016. The study results suggested vonapanitase may improve both fistula use for hemodialysis and secondary patency (time to fistula abandonment), which are the co-primary endpoints in Proteon's ongoing Phase 3 PATENCY-2 clinical trial. The PATENCY-2 clinical trial is fully enrolled and top-line data is expected in March 2019.

PATENCY-1 evaluated the safety and efficacy of a single dose of vonapanitase in patients with chronic kidney disease (CKD) undergoing surgical creation of a radiocephalic arteriovenous fistula for hemodialysis. The randomized, double-blind, placebo-controlled clinical trial enrolled 313 treated patients at 31 medical centers in the United States. Patients were followed for up to one year.

As previously reported, PATENCY-1 did not meet its primary endpoint of improved primary unassisted patency, compared to placebo (p=0.254). However, for its pre-specified secondary endpoint, the trial showed a 34% reduction in the risk of secondary patency loss over one year, compared to placebo (p=0.048). Additionally, the trial showed a 45% relative increase in fistula use for hemodialysis, compared to placebo (p=0.006), which was another pre-specified endpoint.

"While a functioning arteriovenous fistula remains the gold standard for hemodialysis vascular access, failure rates remain unacceptably high, resulting in increased morbidity and mortality and higher health care costs," said Anthony J. Bleyer, M.D., Professor, Nephrology, Wake Forest Baptist Health, and lead author of the publication. "The PATENCY-1 results are promising because of the improvement in fistula use and secondary patency, two clinically meaningful endpoints for patients, their families, and their physicians. I look forward to the results of the ongoing PATENCY-2 trial, in which fistula use and secondary patency are the co-primary endpoints."

The PATENCY-1 results were the basis for Proteon's request to the U.S. Food and Drug Administration (FDA) for Breakthrough Therapy designation, which the FDA granted in May 2017 for increasing arteriovenous fistula secondary patency and use for hemodialysis. The FDA awards Breakthrough Therapy designations to expedite the development and review of investigational drugs that are intended to address a serious or life-threatening condition when preliminary clinical evidence indicates that the drug may offer a substantial improvement over available therapies on one or more clinically significant endpoints.

Based on the results of PATENCY-1, and following discussions with the FDA, Proteon amended the protocol for the PATENCY-2 trial, reordering the endpoints and establishing fistula use for hemodialysis and secondary patency as co-primary endpoints. If PATENCY-2 is successful in showing statistical significance ($p \leq 0.05$) for both of the co-primary endpoints, Proteon expects to file a Biologics License Application (BLA) with the FDA in the second half of 2019 and a Marketing Authorization Application (MAA) with the European Medicines Agency (EMA) in the first half of 2020.

About Chronic Kidney Disease, Hemodialysis and Vascular Access

In the most severe stage of chronic kidney disease (CKD), also known as kidney failure, the kidneys can no longer function to sustain life. The majority of patients with kidney failure undergo chronic hemodialysis, which requires a high-flow vascular access to repeatedly connect the patient's bloodstream to a hemodialysis machine for this life-saving treatment. The preferred form of vascular access for hemodialysis is a radiocephalic arteriovenous fistula, created when a surgeon connects a vein to an artery in the lower arm, resulting in a substantial increase in blood flow and vein dilation.

About Vonapanitase

Vonapanitase is an investigational drug intended to improve hemodialysis vascular access outcomes. Vonapanitase is applied in a single administration and is currently being studied in a Phase 3 clinical trial in patients with chronic kidney disease (CKD) undergoing surgical creation of a radiocephalic arteriovenous fistula for hemodialysis. Vonapanitase has received Breakthrough Therapy, Fast Track and Orphan Drug designations from the FDA, and Orphan Medicinal Product designation from the European Commission, for hemodialysis vascular access indications. In addition, vonapanitase may have other surgical and endovascular applications in diseases or conditions in which vessel injury leads to blockages in blood vessels and reduced blood flow. Proteon is currently conducting a Phase 1 clinical trial of vonapanitase in patients with peripheral artery disease (PAD).

About Proteon Therapeutics

Proteon Therapeutics is committed to improving the health of patients with kidney and vascular diseases through the development of novel, first-in-class therapeutics. Proteon's lead product candidate, vonapanitase, is an investigational drug intended to improve hemodialysis vascular access outcomes. Proteon has completed enrollment in PATENCY-2, a Phase 3 clinical trial evaluating vonapanitase in patients with CKD undergoing surgical creation of a radiocephalic arteriovenous fistula for hemodialysis. Proteon is also evaluating vonapanitase in a Phase 1 clinical trial in patients with PAD. For more information, please visit www.proteontx.com.

Cautionary Note Regarding Forward-Looking Statements

This press release contains statements that are, or may be deemed to be, "forward-looking statements" as defined in the Private Securities Litigation Reform Act of 1995. In some cases, these forward-looking statements can be identified by the use of forward-looking terminology, including the terms "estimates," "anticipates," "expects," "plans," "intends," "may," or "will," in each case, their negatives or other variations thereon or comparable terminology, although not all forward-looking statements contain these words. These statements, including the number of patients to be enrolled in and the timing of enrollment in the Company's ongoing Phase 1 clinical trial of vonapanitase in patients with PAD, when the Company expects to release

top-line data from the PATENCY-2 trial, whether and when the Company may submit a Biologics License Application in the United States or Marketing Authorization Application in Europe, the effect or benefit of vonapanitase in patients with CKD, whether vonapanitase improves fistula use for hemodialysis or secondary patency, the potential surgical and endovascular applications for vonapanitase, including PAD, and those relating to future events or our future financial performance or condition, involve substantial known and unknown risks, uncertainties and other important factors that may cause our actual results, levels of activity, performance or achievements to differ materially from those expressed or implied by these forward-looking statements. These risks, uncertainties and other factors, including whether our cash resources will be sufficient to fund the Company's operating expenses and capital expenditure requirements for the period anticipated; whether data from early nonclinical or clinical studies will be indicative of the data that will be obtained from future clinical trials; whether vonapanitase will advance through the clinical trial process on the anticipated timeline and warrant submission for regulatory approval; whether such a submission would receive approval from the U.S. Food and Drug Administration or equivalent foreign regulatory agencies on a timely basis or at all; and whether the Company can successfully commercialize and market its product candidates, are described more fully in our Annual Report on Form 10-K for the year ended December 31, 2017, as filed with the Securities and Exchange Commission ("SEC") on March 14, 2018, and the Company's subsequent Quarterly Reports on Form 10-Q and Current Reports on Form 8-K, as filed with the SEC, particularly in the sections titled "Risk Factors" and "Management's Discussion and Analysis of Financial Condition and Results of Operations." In light of the significant uncertainties in the Company's forward-looking statements, no person should place undue reliance on these statements or regard these statements as a representation or warranty by the Company or any other person that the Company will achieve its objectives and plans in any specified time frame, or at all. The forward-looking statements contained in this press release represent the Company's estimates and assumptions only as of the date of this press release and, except as required by law, the Company undertakes no obligation to update or revise publicly any forward-looking statements, whether as a result of new information, future events or otherwise after the date of this press release.

Investor Contact
George Eldridge, Proteon Therapeutics, Senior Vice President and Chief Financial Officer
781-890-0102
geldridge@proteontherapeutics.com

Media Contact
Ann Stanesa, Ten Bridge Communications
617-230-0347
proteon@tenbridgecommunications.com

New Proteon Logo - full color RGB.jpg

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