Proteon Therapeutics Receives FDA Breakthrough Therapy Designation for Vonapanitase

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WALTHAM, Mass., May 10, 2017 (GLOBE NEWSWIRE) -- Proteon Therapeutics, Inc. (Nasdaq:PRTO), a company developing novel, first-in-class therapeutics to address the medical needs of patients with kidney and vascular diseases, today announced that its investigational treatment, vonapanitase, has received Breakthrough Therapy designation from the U.S. Food and Drug Administration (FDA) for increasing arteriovenous fistula secondary patency (i.e., survival of the fistula without abandonment) and use for hemodialysis in patients on or expected to initiate hemodialysis.

Secondary patency and fistula use for hemodialysis are the co-primary endpoints of Proteon's ongoing pivotal Phase 3 clinical trial, PATENCY-2. As previously announced, the FDA has confirmed that the PATENCY-2 trial together with data from previously completed studies would provide the basis for a Biologics License Application (BLA) submission as a single pivotal study if PATENCY-2 is successful in showing statistical significance ($p \leq 0.05$) on each of the co-primary endpoints.

The FDA awards Breakthrough Therapy designations to expedite the development and review of drugs that are intended to address a serious or life-threatening condition and preliminary clinical evidence indicates that the drug may offer a substantial improvement over available therapies on one or more clinically significant endpoints. Proteon's Breakthrough Therapy designation is supported by data from PATENCY-1, the Company's first Phase 3 clinical trial evaluating vonapanitase in patients with chronic kidney disease (CKD) undergoing surgical creation of a radiocephalic arteriovenous fistula. In that study, vonapanitase demonstrated clinically meaningful improvements in secondary patency and use for hemodialysis, although it did not meet the primary endpoint of improving primary patency.

"We believe the decision by the FDA to grant vonapanitase a Breakthrough Therapy designation speaks to the clinical importance of fistula survival and use for hemodialysis to patients with chronic kidney disease," said Timothy Noyes, President and Chief Executive Officer of Proteon Therapeutics. "Our productive ongoing dialogue with the FDA has helped to create a clear path forward for vonapanitase, and we look forward to continuing to work closely with the FDA to expedite our development efforts for this important program."

Enrollment in the PATENCY-2 trial is expected to complete in the first quarter of 2018 and Proteon expects to report top-line data in the first quarter of 2019. Proteon also expects to submit a BLA to the FDA in 2019.

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 forearm, 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 program in patients with 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 is currently enrolling patients 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 of vonapanitase 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." 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 timing of enrollment in the PATENCY-2 trial, when the Company expects to report top-line data from the PATENCY-2 trial, whether and when we may submit a BLA, whether additional studies will be necessary to support a BLA submission as a single pivotal trial, the potential treatment of renal and vascular diseases with vonapanitase, the effect or benefit of vonapanitase in patients with CKD, whether vonapanitase improves secondary patency or fistula use for hemodialysis and the clinical importance of these endpoints, 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 our 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 we can successfully commercialize and market our product candidates, are described more fully in our Annual Report on Form 10-K for the year ended December 31, 2016, as filed with the Securities and Exchange Commission ("SEC") on March 16, 2017, and our 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 our forward-looking statements, you should not place undue reliance on these statements or regard these statements as a representation or warranty by us or any other person that we will achieve our objectives and plans in any specified time frame, or at all. The forward-looking statements contained in this press release represent our estimates and assumptions only as of the date of this press release and, except as required by law, we undertake 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.

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