Proteon Therapeutics Announces Full-Year 2016 Financial Results and Changes to the Ongoing Phase 3 PATENCY-2 Clinical Trial

March 16, 2017

- Efficacy endpoints reordered and co-primary endpoints established -
- Ongoing trial, if successful, expected to serve as single pivotal study for BLA submission -

- Conference Call Scheduled for 8:00 AM ET -

WALTHAM, Mass., March 16, 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 its financial results for the year ended December 31, 2016, and provided an update on the PATENCY-2 clinical trial and other recent business highlights.

“The results from PATENCY-1 provided us critical insights into studying vonapanitase that have allowed us to strengthen the PATENCY-2 trial,” said Timothy Noyes, President and Chief Executive Officer of Proteon Therapeutics. “We are encouraged by the high degree of engagement by the FDA and appreciate their continued guidance with respect to our ongoing Phase 3 trial.”

Clinical Trial Update for PATENCY-2

Important changes to PATENCY-2, the second Phase 3 clinical study of investigational vonapanitase. After announcing top-line results from the first Phase 3 clinical trial, PATENCY-1, in December 2016, Proteon started discussions with the U.S. Food and Drug Administration, or FDA, regarding changes to the PATENCY-2 trial. Following the Company’s review of the complete data sets from the PATENCY-1 trial and discussions with the FDA, Proteon amended the protocol for the PATENCY-2 trial as follows:

- The protocol amendment reordered the existing endpoints for the PATENCY-2 trial, establishing secondary patency and fistula use for hemodialysis as co-primary endpoints.

  - Secondary patency is defined as the length of time from surgical creation until fistula abandonment (final failure), the same definition used in PATENCY-1 in which secondary patency served as the secondary endpoint. In PATENCY-1, vonapanitase-treated patients experienced a 34% reduction in the risk of secondary patency loss over one year, compared to placebo (p=0.048). At the end of one year, 74% of vonapanitase-treated patients maintained secondary patency, compared to 61% of placebo-treated patients.

  - Use for hemodialysis is defined as use of the fistula for hemodialysis for at least 90 days or, if hemodialysis was not initiated at least 90 days prior to the patient’s last visit, for at least 30 days prior to the patient’s last visit and in use at the patient’s last visit. This is the same definition used in PATENCY-1. In PATENCY-1, 64% of vonapanitase-treated patients used their fistula for hemodialysis, compared to 44% of placebo-treated patients (p=0.006), a 45% relative increase.

- The protocol amendment also increased the planned enrollment for this trial from 300 to 500 patients, which provides greater than 90% power to detect the differences observed in the PATENCY-1 trial with a p-value ≤0.05 for each of the co-primary endpoints.

- Based on the Company’s interactions with the FDA, Proteon believes that, if the PATENCY-2 trial is successful in showing statistical significance (p≤0.05) on each of the co-primary endpoints, the PATENCY-2 trial together with data from previously completed studies would provide the basis for a Biologics License Application, or BLA, submission as a single pivotal study, in which case no additional studies would need to be conducted.

As of February 28, 2017, Proteon had enrolled 315 patients in PATENCY-2 at approximately 40 centers in the U.S. and Canada. Proteon expects to complete enrollment in the PATENCY-2 trial during the fourth quarter of 2017 and to report top-line data in the fourth quarter of 2018. If the PATENCY-2 trial is successful, Proteon expects to submit a BLA in 2019.

2016 Highlights

Enrollment continues according to plan in PATENCY-2. PATENCY-2 is a multicenter, randomized, double-blind, placebo-controlled study expected to enroll 500 patients in the United States and Canada with chronic kidney disease (CKD) undergoing surgical creation of a radiocephalic arteriovenous fistula for hemodialysis. In February 2017, the Company achieved its guidance from early 2016 to enroll 300 patients by the end of the first quarter of 2017. Full enrollment of 500 patients is expected in the fourth quarter of this year. The study’s co-primary endpoints are secondary patency and fistula use for hemodialysis, each of which demonstrated improvements in PATENCY-1.

Topline clinical results announced in December 2016 for PATENCY-1, the first Phase 3 clinical study of vonapanitase. PATENCY-1 was a multicenter, randomized, double-blind, placebo-controlled study, which enrolled 313 patients in the United States with CKD undergoing surgical creation of a radiocephalic fistula for hemodialysis. While the trial did not show statistical significance for the primary efficacy endpoint, primary unassisted patency, vonapanitase demonstrated improvements in other efficacy endpoints, including secondary patency and fistula use for hemodialysis.

PATENCY-1 clinical results were presented in February 2017 at the American Society of Diagnostic and Interventional Nephrology 13th Annual Scientific meeting. The presentation of Phase 3 results titled, “PATENCY-1: Phase 3 Outcomes of Vonapanitase on Radiocephalic AVF Outcomes,”

was given by Timmy Lee, M.D., Associate Professor of Medicine in the Division of Nephrology at the University of Alabama at Birmingham and an investigator in PATENCY-1. The oral presentation, which detailed the previously announced study results, was given February 11, 2017.

The Company initiated enrollment in a Phase 1 clinical study of vonapanitase in patients with peripheral artery disease (PAD). The multicenter, randomized, double-blind, placebo-controlled Phase 1 dose escalation study is expected to enroll in 2017 up to 24 symptomatic PAD patients being treated with balloon angioplasty of an artery below the knee and to follow each patient for up to seven months. Immediately following successful angioplasty, vonapanitase or placebo is delivered to the arterial wall using the Mercator MedSystems Bullfrog® Micro-Infusion Catheter. The primary outcome measure of the study will be safety and the secondary outcome measure will be technical feasibility of drug delivery via the catheter.

Board of Directors strengthened with the addition of commercial and executive expertise. In the fourth quarter of 2016, the Company appointed Paul J. Hastings as Chairman of its Board of Directors. Mr. Hastings has over three decades of operations experience in the biopharmaceutical industry, including roles as Chief Executive Officer at multiple public companies. Mr. Hastings is President and Chief Executive Officer of OncoMed Pharmaceuticals and Chairman of its Board since 2013.

Key Milestones for 2017

- Complete enrollment of 500 patients in PATENCY-2 in the fourth quarter of 2017.
- Enroll 24 patients in the PAD Phase 1 trial before the end of 2017.

Upcoming Events

- Presentation at the (i) 10th Congress of the Vascular Access Society April 5-8 in Ljubljana, Slovenia (ii) National Kidney Foundation 2017 Spring Clinical Meetings April 18-22 in Orlando, FL and (iii) The Charing Cross Symposium (CX 2017) April 25-28 in London, England.
- Presentations at the (i) Oppenheimer 27th Annual Healthcare Conference March 21-22 in New York City, NY, (ii) Deutsche Bank 42nd Annual Health Care Conference May 3-4 in Boston, MA and (iii) JMP Securities Life Science Conference June 20-21 in New York City, NY.

Full-Year 2016 Financial Results

Cash position: Cash, cash equivalents and available-for-sale investments totaled $41.3 million as of December 31, 2016, compared to $65.3 million as of December 31, 2015. The decrease was driven by operational costs for 2016.

R&D expenses: Research and development expenses for 2016 were $18.9 million as compared to $12.4 million for 2015. The increase in R&D expenses was due primarily to increased expenses for manufacturing pre-validation and validation efforts; increased external clinical expenses related to ongoing radiocephalic AVF Phase 3 clinical trials and our PAD Phase 1 clinical trials; and increased personnel costs.

G&A expenses: General and administrative expenses for 2016 were $9.8 million as compared to $8.5 million for 2015. The increase in G&A expenses was due primarily to higher personnel costs.

Other expense: Other expense for 2016 was $14 thousand as compared to $0.7 million for 2015. Other expense in 2016 and 2015 included non-cash changes in the Swiss Franc denominated currency the Company held as of December 31, 2016 and 2015 and the fair value associated with the forward foreign currency contracts the Company entered into in June 2015.

Net loss: Net loss for 2016 was $28.5 million as compared to $21.4 million for 2015. Net loss included stock-based compensation expense of $3.3 million for 2016 and $2.2 million for 2015.

Financial guidance: The Company expects that its cash, cash equivalents and available-for-sale investments will be sufficient to fund its operations into the third quarter of 2018.

Conference Call and Webcast regarding Clinical Trial Update for PATENCY-2

Proteon is hosting a webcast and conference call today, March 16, at 8:00 a.m. ET to discuss changes to the PATENCY-2 trial. To access the conference call, please dial (844) 263-8297 (U.S.) or (478) 219-0006 (international) with Conference ID # 89059698. A live, listen-only webcast will also be accessible on the Investors & Media page of www.proteontx.com. A replay of the conference call will be available for two weeks on the Proteon website or by dialing (855) 859-2056 (U.S.) or (404) 537-3406 (international) and using Conference ID # 89059698.

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 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 evaluating vonapanitase in patients with CKD undergoing surgical creation of a radiocephalic arteriovenous fistula. Proteon is currently enrolling patients in a Phase 3 clinical trial, PATENCY-2. 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." 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 and planned clinical trials of vonapanitase, when the Company expects to report top-line data from the PATENCY-2 trial, whether and when we may submit a BLA in the United States, whether additional studies will be necessary to support a BLA submission as a single pivotal trial, whether and when we may be able to successful complete drug substance validation runs, the potential treatment of renal and vascular diseases with vonapanitase, the effect or benefit of vonapanitase in patients with CKD, whether vonapanitase improves fistula patency or use for hemodialysis, the potential surgical and endovascular applications for vonapanitase, including PAD, the sufficiency of the Company’s cash, cash-equivalents and available-for-sale investments to fund the Company’s operations into the third quarter of 2018, 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.

Proteon Therapeutics, Inc.
Consolidated Balance Sheet Data
(In thousands)

	December 31, 2016	2015
Cash, cash equivalents and available-for-sale investments	$ 41,317	$ 65,263
Prepaid expenses and other current assets	1,438	1,345
Property and equipment, net and other non-current assets	765	930
Total assets	**$ 43,520**	**$ 67,538**
Accounts payable and accrued expenses	$ 5,079	$ 3,596
Other liabilities	-	537
Common stock and additional paid-in-capital	198,218	194,667
Accumulated deficit and accumulated other comprehensive loss	(159,777)	(131,262)
Total liabilities and stockholders’ deficit	**$ 43,520**	**$ 67,538**

Proteon Therapeutics, Inc.

Condensed Consolidated Statements of Operations
(in thousands, except share and per share data)

	Year Ended December 31,		
	2016	**2015**	**2014**
Revenue	$ -	$ -	$ 2,948
Operating expenses:			
Research and development	18,869	12,381	6,432
General and administrative	9,836	8,489	4,096
Total operating expenses	28,705	20,870	10,528
Loss from operations	(28,705)	(20,870)	(7,580)
Other income (expense):			
Interest income (expense)	193	144	(833)
Other (expense) income	(14)	(651)	5,071
Total other (expense) income	179	(507)	4,238
Net loss	$ (28,526)	$ (21,377)	$ (3,342)
Net loss per share attributable to common stockholders - basic and diluted	$ (1.72)	$ (1.30)	$ (3.16)
Weighted-average common shares outstanding - basic and diluted	16,561,799	16,464,123	3,064,507

Supplemental disclosure of stock-based compensation expense and loss from currency forward contracts:

Included in operating expenses, above, are the following amounts for non-cash stock based compensation expense:			
Research and development	$ 1,114	$ 650	$ 114
General and administrative	2,229	1,514	345
Total	$ 3,343	$ 2,164	$ 459
Included in other expense, above, are the following amounts from forward foreign currency contracts:			
Realized losses from forward foreign currency contracts	$ (61)	$ (52)	$ -
Unrealized losses from forward foreign currency contracts	127	(537)	-
Total	$ 66	$ (589)	$ -

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