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Data From Prothena's Ongoing Phase 1/2 Clinical Trial of NEOD001 in AL Amyloidosis Published in the Journal of Clinical Oncology

Interim Results Previously Presented at Meetings of the American Society for Clinical Oncology and the European Hematology Association

DUBLIN, Feb. 09, 2016 (GLOBE NEWSWIRE) -- Prothena Corporation plc (NASDAQ:PRTA), a late-stage clinical biotechnology company focused on the discovery, development and commercialization of novel protein immunotherapies, today announced that interim data from its Phase 1/2 clinical study of NEOD001 in patients with AL Amyloidosis and persistent organ dysfunction have been published in the Journal of Clinical Oncology. The data appeared online in an article titled "First-in-Human Phase 1/2 Study of NEOD001 in Patients With Light Chain Amyloidosis and Persistent Organ Dysfunction."

The published data, which were previously presented in oral presentations at the American Society for Clinical Oncology Annual Meeting and the European Hematology Association Congress, showed that monthly infusions of NEOD001 were safe and well tolerated and that patients showed improvements in functional biomarkers predictive of improvement of disease and survival. Data from the ongoing expansion cohort of this study are expected in the second quarter of 2016.

"The published data provide strong support for the continued development of NEOD001 in AL amyloidosis, and we hope that through Prothena's ongoing clinical development efforts, we may one day soon have an approved immunotherapy treatment that positively impacts organ function for this difficult-to-treat and deadly disease," said Morie A. Gertz, M.D., of the Mayo Clinic, principal investigator of the study and lead author of the manuscript.

A total of 27 patients with AL amyloidosis and persistent organ dysfunction were enrolled in seven dose cohorts in the dose escalation portion of the study. Cardiac and renal responses were exploratory endpoints.

Of the 14 cardiac-evaluable patients, 57 percent met the criteria for cardiac response, which compares favorably to the expected cardiac best response rate of 26.5 percent from historical data in patients treated solely with off-label standard of care (Comenzo, et al., Leukemia. 2012; 26:2317-2325). Forty-three percent of patients had stable disease. In cardiac responders, the median NT-proBNP, a clinical biomarker used to predict disease progression and mortality, decreased from 1768.5 pg/mL at baseline to 1054 pg/mL at the best response assessment. The mean decrease from baseline was 890 pg/mL, which represented a 48 percent reduction and was statistically significant ($P<0.008$). NT-proBNP decline for responders significantly correlated with increased number of NEOD001 infusions.

Of the 15 renal-evaluable patients, 60 percent met the criteria for renal response which compares favorably to the expected renal best response of approximately 24 percent from historical data in patients treated solely with off-label standard of care (Palladini, et al., Blood. 2014 124:2325-2332). Forty percent of patients had stable disease. Increased levels of proteinuria and decreased eGFR predict faster progression to dialysis whereas decreased levels of proteinuria in the absence of eGFR worsening predict delayed time to dialysis. In the responders, the median proteinuria value decreased from 4834 mg/24 hours at baseline to 1647 mg/24 hours at the best response assessment. The mean decrease from baseline was —2647 mg/24 hours, which represented a 62 percent decrease and was statistically significant ($P<0.004$).

"The positive results from our Phase 1/2 study of NEOD001 in patients with AL amyloidosis suggest that NEOD001 may effectively recruit the immune system to clear the circulating soluble and deposited insoluble proteins that cause organ dysfunction in this progressive and life-threatening condition," said Dale Schenk, Ph.D., President and Chief Executive Officer of Prothena. "Based on these data, Prothena has initiated The VITAL Amyloidosis Study, a global registration study for NEOD001, and is planning to initiate the PRONTO study, which will evaluate cardiac response as assessed by changes in NT-proBNP, a cardiac functional biomarker that has been shown to be highly predictive of survival in patients with AL amyloidosis."

Monthly infusions of NEOD001 were safe and well tolerated. There were no drug-related serious adverse events (AEs), discontinuations due to drug-related AEs, dose-limiting toxicities or antidrug antibodies reported in the study. The most frequently reported AEs were fatigue, upper respiratory tract infection, cough, and dyspnea. The recommended dose level was 24 mg/kg and the pharmacokinetic profile of the drug support intravenous dosing once every 28 days. As previously reported, there was one patient death that was determined to be unrelated to NEOD001.

About NEOD001

NEOD001 is a humanized monoclonal antibody that specifically targets the circulating soluble amyloid and deposited insoluble amyloid that accumulates in both the AL and AA forms of amyloidosis. NEOD001 received Fast Track designation from the FDA in December 2014. There are three clinical trials for NEOD001 in patients with AL amyloidosis. The multi-center Phase 1/2 clinical trial is evaluating the safety, tolerability, pharmacokinetics and immunogenicity of NEOD001 in patients with AL amyloidosis and persistent organ dysfunction. The trial is also evaluating exploratory biomarkers for cardiac and renal function as well as peripheral neuropathy. The VITAL Amyloidosis Study, a double-blind, placebo-controlled, global Phase 3 registrational trial, will evaluate NEOD001 in newly-diagnosed, treatment-naïve patients with AL amyloidosis, and will assess a composite of all-cause mortality or cardiac hospitalizations in addition to biomarker, functional and quality of life endpoints. The PRONTO trial, a double-blind, placebo-controlled, global Phase 2b registration-directed trial, will evaluate NEOD001 in previously-treated patients with AL amyloidosis and persistent cardiac dysfunction, and will assess NT-proBNP best response over 12 months in addition to other biomarker, functional, and quality of life endpoints. More information on the Phase 1/2 trial, The VITAL Amyloidosis Study, and the PRONTO trial is available at www.clinicaltrials.gov.

About AL Amyloidosis

Systemic amyloidoses are a complex group of progressive diseases caused by tissue deposition of misfolded proteins that result in progressive organ damage. The most common type, AL amyloidosis or primary amyloidosis, involves a hematological disorder caused by plasma cells that produce misfolded immunoglobulin light chain resulting in deposits of abnormal AL protein (amyloid) in the tissues and organs of individuals with this disease. There are no approved treatments for AL amyloidosis, and none that directly target potentially toxic forms of the AL protein. AL amyloidosis is a rare disorder and it is estimated that about 30,000 to 45,000 patients in the U.S. and Europe suffer from this disease. Both the causes and origins of AL amyloidosis remain poorly understood. For more information on AL amyloidosis, please visit the websites of the Amyloidosis Support Group and the Amyloidosis Foundation.

About Prothena

Prothena Corporation plc is a late-stage clinical biotechnology company focused on the discovery, development and commercialization of novel protein immunotherapies for the potential treatment of diseases that involve amyloid or cell adhesion. The Company is developing antibody-based product candidates that target a number of potential indications including AL amyloidosis (NEOD001), Parkinson's disease and other related synucleinopathies (PRX002), and psoriasis and other inflammatory diseases (PRX003).

For more information, please visit the Company's web site at www.prothena.com.

Forward-looking Statements

This press release contains forward-looking statements. These statements relate to, among other things, the potential clinical benefits of NEOD001; the initiation of the PRONTO clinical trial; the PRONTO trial design; and the expected timing for announcing data from the expansion cohort of the Phase 1/2 clinical trial. These statements are based on estimates, projections and assumptions that may prove not to be accurate, and actual results could differ materially from those anticipated due to known and unknown risks, uncertainties and other factors, including but not limited to the risks, uncertainties and other factors described in the "Risk Factors" sections of our Annual Report on Form 10-K filed with the Securities and Exchange Commission (SEC) on March 13, 2015 and our subsequent Quarterly Reports on Form 10-Q filed with the SEC. Prothena undertakes no obligation to update publicly any forward-looking statements contained in this press release as a result of new information, future events or changes in Prothena's expectations.

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