March 12, 2018

Prothena to Present a Broad Range of Scientific and Health Outcomes Data at the 16th International Symposium on Amyloidosis

DUBLIN, Ireland, March 12, 2018 (GLOBE NEWSWIRE) -- Prothena Corporation plc (Nasdaq:PRTA), a late-stage clinical biotechnology company focused on the discovery, development and commercialization of novel therapies in the neuroscience and orphan categories, today announced that it will present new research in both AL and ATTR amyloidosis at the 16th International Symposium on Amyloidosis (ISA), to be held March 26 to 29 in Kumamoto, Japan.

"Prothena is committed to conducting, presenting and publishing innovative research that advances our understanding of the underlying pathophysiology in systemic amyloidoses to support the development of potential treatments for patients," said Wagner Zago, Ph.D., Chief Scientific Officer of Prothena. "We look forward to presenting a broad range of our research in both AL and ATTR amyloidosis at ISA, including preclinical research that provides new insights into the mechanism of action for NEOD001, a potential treatment for AL amyloidosis and its ability to recognize a cryptic epitope that is exposed on both kappa and lambda light chains from even the earliest stages of the misfolding and aggregation process. In addition, we will share data that further describes PRX004, our investigational antibody for the treatment of ATTR amyloidosis, including a highly specific assay we have developed to measure the misfolded forms of TTR protein present in plasma of patients with hereditary ATTR amyloidosis."

New research will be presented demonstrating that NEOD001 binds to both soluble and insoluble aggregated kappa and lambda light chains and recognizes an epitope that is exposed during even the earliest stages of abnormal light chain misfolding and aggregation involved in AL amyloidosis. In addition, preclinical data will be presented that provides further insight into how the toxicity induced by light-chains can modulate NT-proBNP production in AL amyloidosis, which is unique from other forms of heart failure, and offers support for the relationship that has been reported between lowering of NT-proBNP and improved survival in patients with AL amyloidosis. These presentations will occur in the following poster sessions:

(Abstract #PC095) NEOD001 Binds a Wide Repertoire of Light Chain Sequences and Aggregation States Found in AL Amyloidosis

- Presenter: Wagner Zago, PhD, Chief Scientific Officer, Prothena
- Date and Time: March 28, 1:00-2:00 PM JST
- Location: Poster Hall

(Abstract #PB069) Aggregated Light Chain Increases Brain Natriuretic Peptide Production and Induces Oxidative Stress Response in Cardiomyocytes

- Presenter: Stephen J. Tam, PhD, Senior Scientist, Prothena
- Date and Time: March 27, 1:00-2:00 PM JST
- Location: Poster Hall

Additional research related to PRX004 will be presented, including a proprietary assay that specifically detects circulating misfolded-hATTR in plasma across multiple TTR mutations using a TTR antibody that binds to an epitope uniquely exposed on misfolded TTR but hidden in the native tetramer. Such an assay has the potential to be used as a diagnostic that can detect misfolded TTR in the plasma of patients with hereditary ATTR amyloidosis. Additional preclinical research will be presented showing that conformation-specific antibodies target misfolded TTR and immune mediated clearance of amyloid through phagocytosis. These presentations will occur in the following oral and poster sessions:

(Abstract #PB052) Detection of Misfolded Forms of TTR in Plasma from Patients with Hereditary ATTR Using Conformation-specific TTR Antibodies

- Presenter: Jeffrey N. Higaki PhD, Director, Biochemistry, Prothena
- Date and Time: March 27, 8:20-8:30 AM JST (oral)
- Date and Time: March 27, 1:00-2:00 PM JST (poster)
- Location: Poster Hall

(Abstract #PC009) In Vivo Target Engagement and Phagocytosis of Aggregated TTR by a Conformation-specific

TTR Antibody

- Presenter: Jeffrey N. Higaki, PhD, Director, Biochemistry, Prothena
- Date and Time: March 28, 1:00-2:00 PM JST
- Location: Poster Hall

Presentations on Quality of life, health economics and epidemiology research in patients with AL amyloidosis, including real world data elucidating the burden of disease from delayed diagnosis to substantial comorbidities and healthcare resource use, will also be presented in the following poster sessions:

(Abstract #PA085) A Mixed Methods Study of the Journey to Diagnosis Among Patients with Light Chain Amyloidosis

- Presenter: Tiffany P. Quock, PhD, MS, Senior Director, Health Economics and Outcomes Research, Prothena
- Date and Time: March 26, 1:30-2:30 PM JST
- Location: Poster Hall

(Abstract #PA086) Real-world Burden of Comorbidities in Patients with Newly Diagnosed AL Amyloidosis

- Presenter: Tiffany P. Quock, PhD, MS, Senior Director, Health Economics and Outcomes Research, Prothena
- Date and Time: March 26, 1:30-2:30 PM JST
- Location: Poster Hall

(Abstract #PA087) Real-world Healthcare Utilization and Costs in Patients with Newly Diagnosed AL Amyloidosis

- Presenter: Tiffany P. Quock, PhD, MS, Senior Director, Health Economics and Outcomes Research, Prothena
- Date and Time: March 26, 1:30-2:30 PM JST
- Location: Poster Hall

(Abstract #PC070) Epidemiology of AL Amyloidosis in US Commercially Insured Population

- Presenter: Tiffany P. Quock, PhD, MS, Senior Director, Health Economics and Outcomes Research, Prothena
- Date and Time: March 28, 1:00-2:00 PM JST
- Location: Poster Hall

About AL and ATTR Amyloidosis

Systemic amyloidoses are a complex group of diseases caused by tissue deposition of misfolded proteins that result in progressive organ damage.

Amyloid light chain amyloidosis (AL amyloidosis), the most common type, is a rare, progressive, and typically fatal protein misfolding disease caused by extracellular deposition of aggregated immunoglobulin light chains. An excess of light chains prone to misfolding are produced by clonal plasma cells. Soluble toxic aggregates and deposited fibrils (amyloid) lead to progressive failure of vital organs including the heart, kidneys and nervous system, causing significant morbidity and mortality. It is estimated that approximately 30,000 — 45,000 patients in the U.S. and Europe suffer from this disease. There are no approved treatments for AL amyloidosis, although patients may be treated with off-label therapies directed at the plasma cell dyscrasia. There is a large unmet need for therapies that specifically target soluble toxic aggregates and deposited fibrils, thereby improving vital organ function.

Transthyretin amyloidosis (ATTR amyloidosis) is a rare, progressive and often fatal disease characterized by deposition of aggregates of misfolded protein, or amyloid. There are three types of ATTR amyloidosis: hereditary ATTR with cardiomyopathy (hATTR-CM); wild-type ATTR (wtATTR) which occurs sporadically and also involves cardiomyopathy; and hereditary ATTR with polyneuropathy (hATTR-PN). The TTR protein is produced primarily in the liver and in its normal tetrameric form serves as a carrier for thyroxin and vitamin A. In hereditary hATTR-PN and hATTR-CM the body makes a mutant form of the TTR protein. There are more than 100 reported types of TTR mutations that promote amyloid fibril formation, which most commonly affect the heart (hATTR-CM) and nervous system (hATTR-PN). Wild-type ATTR (wtATTR) is similar to hereditary ATTR except that the protein that is deposited is the misfolded, non-mutated transthyretin protein.

For more information on AL and ATTR amyloidosis, please visit the websites of the Amyloidosis Support Groups, The Amyloidosis Research Consortium, and the Amyloidosis Foundation.

About NEOD001

NEOD001 is an investigational first-in-class antibody that specifically targets disease-causing misfolded light chain aggregates in AL amyloidosis. There are two ongoing global clinical studies for NEOD001. The PRONTO study, a global, Phase 2b, double-blind, placebo-controlled, registration-directed study, will evaluate NEOD001 vs. placebo in previously-treated patients with AL amyloidosis and persistent cardiac dysfunction, and will assess best response over 12 months of the cardiac biomarker NT-proBNP, defined by the consensus criteria of NT-proBNP change, in addition to other biomarker, quality of life and functional endpoints. The VITAL Amyloidosis Study, a global, Phase 3, double-blind, placebo-controlled, registrational study, is evaluating NEOD001 vs. placebo in newly-diagnosed, treatment-naïve patients with AL amyloidosis and cardiac dysfunction, with both arms of the study receiving standard of care. The VITAL study will assess a composite endpoint of all-cause mortality or cardiac hospitalizations in addition to biomarker, quality of life and functional endpoints. More information on the PRONTO study and The VITAL Amyloidosis Study is available at www.clinicaltrials.gov, by searching NCT #02632786 for PRONTO, and NCT #02312206 for VITAL or www.clinicaltrialsregister.eu, by searching EudraCT #2015-004318-14 for PRONTO, and EudraCT #2014-003865-11 for VITAL.

About PRX004

PRX004 is a monoclonal antibody designed to specifically target and clear the misfolded forms of the amyloid TTR protein found in both hereditary (hATTR-CM and hATTR-PN) and wild type (wtATTR) ATTR amyloidosis, and leave the native form of the protein unaffected. Currently in preclinical development, Prothena plans to advance PRX004 into the clinic as a potential therapy for ATTR amyloidosis.

About Prothena

Prothena Corporation plc is a global, late-stage clinical biotechnology company establishing fully integrated research, development and commercial capabilities and focused on advancing new therapies in the neuroscience and orphan categories. Fueled by its deep scientific understanding built over decades of research in protein misfolding, Prothena seeks to fundamentally change the course of grave or currently untreatable diseases associated with this biology. Prothena's pipeline of antibody therapeutic candidates targets a number of indications including AL amyloidosis (NEOD001), Parkinson's disease and other related synucleinopathies (PRX002/RG7935) and ATTR amyloidosis (PRX004). The Company continues to advance additional discovery programs against targets including tau, Aβ (Amyloid beta) and ALECT2 where its deep scientific understanding of disease pathology can be leveraged. For more information, please visit the Company's website at www.prothena.com.

Forward-looking Statements

This press release contains forward-looking statements. These statements relate to, among other things, the proposed mechanism of action of NEOD001; the relationship between NT-proBNP and survival in patients with AL amyloidosis; the proposed mechanism of action of PRX004; the potential of our proprietary assay to be used as a diagnostic to detect misfolded TTR in patients with hereditary ATTR amyloidosis; the expected timing of advancing PRX004 into clinical development; and whether we can continue to advance additional discovery programs. 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 February 26, 2018 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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