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## **Prothena Achieves Orphan Drug Status in EU for Lead Program NEOD001**

### **Potential Treatment for Systemic Amyloidoses Also Has US Designation**

DUBLIN, Ireland, Feb. 27, 2013 (GLOBE NEWSWIRE) -- Prothena Corporation plc (Nasdaq:PRTA), a biotechnology company focused on the discovery and development of novel antibodies for the potential treatment of a broad range of diseases, today announced that the European Medicines Agency (EMA) granted orphan designation for its lead program, NEOD001, for the potential treatment of amyloid light-chain (AL) amyloidosis. In 2012, the US Food and Drug Administration (FDA) granted orphan drug status to this program. NEOD001 is a monoclonal antibody that specifically targets the amyloid that accumulates in the organs of patients with the disease.

"AL amyloidosis is the most common and pathogenic form of systemic amyloidoses, and securing orphan designation for NEOD001 in the European Union is an important milestone for Prothena and for patients," said Dale Schenk, PhD, Prothena's President and Chief Executive Officer. "NEOD001 targets systemic amyloidoses, diseases in which misfolded proteins accumulate in the body's organs, causing progressive damage and death. We expect to start Phase I clinical trials of NEOD001 in primary amyloidosis patients early this year. If proven effective in clinical trials, our approach has the potential to provide a novel therapy for this orphan disease with significant unmet medical need."

"Orphan drug designations support our global development strategy for NEOD001 and our goal of providing improved therapies for patients with amyloidoses," said Gene Kinney, PhD, Prothena's Chief Scientific Officer and Head of Research and Development. "Our preclinical experience to date with NEOD001 has indicated its potential in this disease area, where limited treatment options exist. The orphan drug designations in the EU and the US provide recognition of the need for therapeutics in this area and help advance the development of potential treatments for patients."

#### **About NEOD001**

NEOD001 is a monoclonal antibody that specifically targets the amyloid that accumulates in both AL and secondary systemic amyloidosis. If proven safe and effective in clinical trials, this approach has the potential to be a first-in-class agent for this orphan disease with a significant unmet medical need. In 2012, NEOD001 was granted orphan drug designation by the FDA, and an Investigational New Drug application for NEOD001 in systemic amyloidosis was filed. Prothena plans to initiate a Phase 1 clinical trial for NEOD001 in this indication in early 2013. NEOD001 is being developed by Onclave Therapeutics, Limited, a wholly-owned subsidiary of Prothena. More information on the planned clinical trial for NEOD001 is available on [clinicaltrials.gov](http://clinicaltrials.gov).

#### **About Amyloidoses**

Systemic amyloidoses are a complex group of 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 AL protein resulting in deposits of abnormal AL protein (amyloid), in the tissues and organs of individuals with AL amyloidosis. There are no currently approved treatments for AL amyloidosis that directly target potentially toxic forms of the AL protein.

#### **About Orphan Drug Designations**

EMA's Orphan Medicinal Product Designation is designed to promote the development of drugs that may provide significant benefit to patients suffering from rare, life-threatening diseases, and provides 10 years of market exclusivity if approved. Similarly, the FDA orphan drug designation is intended to encourage companies to develop therapies for the treatment of diseases that affect fewer than 200,000 individuals in the United States, and allows for fast-track designation and market exclusivity if approved.

#### **About Prothena Corporation**

Prothena Corporation plc (Nasdaq:PRTA) is a biotechnology company focused on the discovery and development of novel antibodies for the potential treatment of a broad range of diseases that involve protein misfolding or cell adhesion, particularly on the discovery and development of potential therapeutic monoclonal antibodies directed specifically to disease-causing proteins. These potential therapies have a broad range of indications, including AL and AA forms of amyloidosis, Parkinson's disease and related synucleinopathies, and novel cell adhesion targets involved in autoimmune disease and metastatic

cancers. For more information, please visit [www.prothena.com](http://www.prothena.com).

## **Forward-looking Statements**

*This press release contains forward-looking statements within the meaning of the Section 27A of the Securities Act of 1933 and Section 21E of the Securities Exchange Act of 1934. These statements relate to, among other things, the status and timing of our products in research, including the timing of Phase I clinical trials, and its effectiveness as a therapy for amyloidoses. These forward-looking statements are identified by their use of terms and phrases such as "anticipate," "believe," "could," "estimate," "expect," "intend," "may," "plan," "predict," "project," "potential," "target," "will" and similar terms and phrases, including references to assumptions. These statements are based on assumptions that may not prove accurate. 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 and uncertainties described in the "Risk Factors" section of the information statement included as an exhibit to our registration statement on Form 10, which has been declared effective by the Securities and Exchange Commission. 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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