

# **ProteoTech is Granted Orphan Drug Designation for Systebryl™ (PTI-110) for the Treatment of AL Amyloidosis**

KIRKLAND, WA, USA, January 12 2015 (BUSINESS NEWSWIRE) – ProteoTech, Inc. ("ProteoTech"), a private biotechnology company focused on the discovery and development of novel small molecule drugs for the treatment of amyloid diseases and misfolded protein disorders, announced today that the FDA has granted orphan drug designation for its lead product Systebryl™ (PTI-110) for the treatment of AL (immunoglobulin light chain) amyloidosis. Systebryl™ (PTI-110) is a drug that has been internally developed at ProteoTech, which targets the insoluble AL amyloid deposits that accumulate in systemic organs (such as the heart and kidney) of patients with this disease.

"Securing orphan designation for Systebryl™ (PTI-110) in the United States for the treatment of AL amyloidosis is a major milestone for ProteoTech. Similar to other drug development programs utilizing ProteoTech's small molecule technology platform, Systebryl™ (PTI-110) is specifically designed to treat a significant unmet medical need," said Roger S. Flugel, Ph.D., ProteoTech's Chief Executive Officer.

"ProteoTech expects to initiate a Phase 1/2 proof-of-concept clinical trial of Systebryl™ (PTI-110) in AL amyloidosis patients later this year. If Systebryl™ (PTI-110) is proven to be safe and effective, this small molecule drug could become a breakthrough treatment for patients with AL amyloidosis, in which there are no current therapies approved specifically for this devastating illness."

"AL amyloidosis is the most common form of systemic amyloid disease. ProteoTech receiving FDA orphan drug designation for Systebryl™ (PTI-110) in the United States is an important advancement for our company, as well as for patients suffering from AL amyloidosis," said Alan D. Snow, Ph.D., ProteoTech's Founder, President and Chief Scientific Officer.

## **About Systebryl™ (PTI-110)**

Systebryl™ (PTI-110) is a novel small molecule drug that specifically targets the amyloid deposits that accumulate in AL and other systemic amyloid diseases. If proven safe and effective in clinical trials, this approach has the potential to be a first-in-class disease-modifying drug for this important rare disease. ProteoTech plans to file an IND (Investigational New Drug) application for Systebryl™ (PTI-110) early this year. ProteoTech plans to then initiate a Phase 1/2 proof-of-concept clinical trial for Systebryl™ (PTI-110) in patients with AL amyloidosis. Systebryl's mechanism of action is unique and is believed to disaggregate and cause the clearance of AL amyloid deposits from the body.

## **About Amyloidosis**

Systemic amyloidosis 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 that

is caused by plasma cells which overproduce misfolded immunoglobulin light chain proteins, resulting in the deposition of insoluble amyloid in the tissues and organs of individuals suffering with AL amyloidosis. Currently, there are no approved treatments for AL amyloidosis that directly act upon the fibrillar and insoluble toxic forms of the AL amyloid protein.

### **About Orphan Drug Designations**

The FDA Orphan Drug Designation is designed to promote the development of drugs that may provide significant benefit to patients suffering from rare diseases. This 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 7-year market exclusivity, if a designated therapy is ultimately approved by the FDA.

### **About ProteoTech, Inc.**

ProteoTech is a privately-held, drug development company that utilizes its amyloid expertise to discover and develop new drugs for treating misfolded protein disorders and amyloid diseases. ProteoTech's lead small molecule drug, Systebryl™ (PTI-110), will soon be entering a Phase 1/2 proof-of-concept clinical trial in patients with AL (immunoglobulin light chain) amyloidosis (a recognized rare or "orphan" disease). Other products in the ProteoTech pipeline include a small molecule drug that is designed to inhibit and stimulate the reduction of insoluble alpha-synuclein aggregate accumulation (involved in Multiple System Atrophy and Parkinson's disease), and small molecule drugs intended to inhibit and reduce tau protein and/or beta-amyloid protein aggregates (involved in Progressive Supranuclear Palsy and Alzheimer's disease), and small molecule drugs intended to inhibit and reduce beta2-microglobulin protein aggregates (involved in dialysis-related amyloidosis). For further information, please visit [www.proteotech.com](http://www.proteotech.com), email [info@proteotech.com](mailto:info@proteotech.com), or telephone +1-425-823-0400.