

## **FOLDRX ANNOUNCES THE INITIATION OF A PHASE I STUDY WITH FIRST CLINICAL CANDIDATE**

*-- First-in-class disease-modifying agent, Fx-1006A, to stabilize protein misfolding in hereditary amyloid disorders --*

October 12, 2005

**Cambridge, MA, October 12, 2005** - FoldRx Pharmaceuticals, Inc. (FoldRx) announced today that it has initiated a Phase I clinical study with its lead candidate, Fx-1006A, a small molecule compound with the potential to treat genetic disorders, such as familial amyloid cardiomyopathy (FAC) and familial amyloid polyneuropathy (FAP). Fx-1006A is a first-in-class, disease-modifying compound that is designed to inhibit the formation of amyloid deposits by preventing the misfolding and deposition of the transthyretin protein (TTR), which is associated with these diseases.

This Phase I trial will be a double-blind, placebo controlled single and multiple dose escalation study, enrolling approximately 55 healthy volunteers. The study will seek to evaluate safety and tolerability of Fx-1006A and also to determine the compound's pharmacologic properties and TTR stabilization activity.

"Protein misfolding has been increasingly recognized as the underlying cause of a number of chronic degenerative diseases. This program provides the first opportunity to evaluate our approach to stabilizing misfolded proteins associated with disease. FAP and FAC, both fatal diseases, are the most common forms of hereditary amyloidoses. Both pathologies have direct links to TTR misfolding and no current drug therapy exists," noted Richard Labaudinière, Ph.D., President and CEO of FoldRx. "The initiation of this clinical trial underscores the rapid progress we have made in the two years since the company's inception."

TTR is a hormone-carrying protein that is produced in the liver and circulates in the blood. In patients with certain genetic mutations, TTR is destabilized and misfolds, resulting in amyloid deposits in various tissues. TTR misfolding is associated with a number of amyloid diseases, which typically occur in patients aged 30 and above. It is believed that stabilization of transthyretin will inhibit further amyloid deposition and stop progression of the disease in FAP and FAC patients.

In FAP, deposition of TTR amyloid occurs in the peripheral nerve tissue and results in sensory neuropathy, starting in the lower extremities. Liver transplantation is currently the only treatment available for these patients. FAC occurs when TTR amyloid deposits infiltrate the heart, leading to progressive heart failure. The predominant mutation in FAC is present in more than 3 percent of individuals of African descent. There are currently no treatments available for this disease.

### **About FoldRx Pharmaceuticals, Inc.**

FoldRx Pharmaceuticals is a development and discovery company focusing on first-in-class disease-modifying small molecule therapeutics to treat diseases of protein misfolding and aggregation (amyloidosis). Protein misfolding is increasingly being recognized as an underlying cause of many chronic degenerative diseases. By applying FoldRx's proprietary expertise in protein folding and its platform for drug and target discovery, the company is building a pipeline, initially for neurodegenerative and cardiovascular conditions. FoldRx's initial pipeline includes a program in advanced pre-clinical development to treat genetic neurologic and cardiovascular disorders, Familial Amyloid Polyneuropathy (FAP) and Familial Amyloid Cardiomyopathy (FAC), and a discovery program in Parkinson's disease, based on its broad, proprietary yeast-based drug discovery platform. For more information on FoldRx, please visit the company's web site at [www.foldrx.com](http://www.foldrx.com).