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**FOR IMMEDIATE RELEASE**

**FOLDRX ANNOUNCES ITS PHASE II/III CLINICAL STUDY WITH LEAD CLINICAL CANDIDATE**

*-- Fx-1006A is being evaluated as a disease-modifying agent in the treatment of Familial TTR Amyloidosis --*

**Cambridge, MA, May 29, 2007** – FoldRx Pharmaceuticals, Inc. (FoldRx) today announced that enrollment is underway in a multinational Phase II/III clinical study with its lead drug candidate, Fx-1006A, for patients suffering from Familial Amyloid Polyneuropathy (FAP), a rare genetic disease that affects approximately 10,000 people worldwide. FAP is a fatal disorder in which amyloid fibrils, caused by the ‘misfolding’ of a protein called transthyretin (TTR), are deposited in peripheral nerve tissues that serve the limbs and organs. FAP results in progressive loss of sensation and motor function, organ failure, pain and weakness. Fx-1006A is designed to stop the progression of this disease by preventing the accumulation of amyloid fibrils caused by the ‘misfolding’ of TTR. Protein folding is the process by which a protein assumes its characteristic functional shape and is essential for normal cellular function.

“There is a significant unmet medical need for patients with this devastating disease,” notes Teresa Coelho, M.D. (Hospital Santo Antonio in Porto, Portugal), one of the worldwide experts on the disease. “This compound has shown activity in stabilizing transthyretin, which represents the first real advance in treating this disease. We are pleased to be participating in this important clinical study to evaluate its potential as a disease-modifying therapy. “

This randomized, double-blind, placebo-controlled, multicenter, multinational Phase II/III study is evaluating the safety and efficacy of orally-administered Fx-1006A in 120 patients worldwide with FAP and a confirmed V30M TTR mutation, the most prevalent disease variant. Participants will undergo an 18-month treatment regimen with once a day dosing. The co-primary endpoints will measure response to treatment at 18 months via the Neuropathy Impairment Score - Lower Limb (NIS-LL) and quality of life, as measured by the Norfolk QOL-DN.

Richard Labaudinière, Ph.D., President and CEO of FoldRx, noted, “We have made rapid progress with Fx-1006A, advancing it from the initiation of a clinical development program into this Phase II/III clinical trial in just over a year. This study will be the first opportunity to test clinical proof-of-concept with our lead candidate. As part of our

program to evaluate this compound, we plan to initiate additional studies in 2008 in patients with other TTR genetic mutations.”

### **About Fx-1006A**

Fx-1006A is a first-in-class, disease-modifying, small-molecule compound that stabilizes wild-type and variant TTR, prevents misfolding and inhibits the formation of TTR amyloid fibrils. The stabilization effect of Fx-1006A has been demonstrated ex-vivo in plasma samples of healthy volunteers and TTR amyloidosis patients. In a dose escalating Phase I study in healthy volunteers, Fx-1006A was found to be safe and well-tolerated. None of the study participants discontinued dosing due to adverse events. Additionally, Fx-1006A demonstrated strong TTR stabilization effects in plasma of participants, even 24 hours after oral administration of the drug. Fx-1006A has orphan drug designation in both the U.S. and European Union (EU) and Fast Track designation in the U.S. for the treatment of FAP. The Fx-1006A development program is evaluating Fx-1006A as a disease modifying agent in the treatment of TTR amyloidosis.

### **About Transthyretin Amyloidosis**

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. Stabilization of transthyretin should inhibit further amyloid deposition and stop progression of diseases such as FAP. In FAP, deposition of TTR amyloid occurs in the peripheral nerve tissue and results in sensorimotor and autonomic neuropathy, starting in the lower extremities. Liver transplantation is currently the only treatment available for these patients.

TTR amyloid deposits also play a key role in another disease, Familial Amyloid Cardiomyopathy (FAC), a disease for which FoldRx is also pursuing therapeutic development. In FAC patients, TTR amyloid fibrils infiltrate the myocardium of the heart, leading to diastolic dysfunction progressing to restrictive cardiomyopathy and heart failure. The predominant mutation in FAC, V122I, is present in nearly four percent of the U.S. African American population.

A mutation in transthyretin is not a prerequisite for the development of transthyretin-associated cardiac amyloidosis. In the elderly, wild-type (normal) transthyretin may become structurally unstable resulting ultimately in the formation of amyloid fibrils, primarily in heart tissues. When this occurs, senile systemic amyloidosis (SSA) is said to be present. There are currently no treatments available for FAC or SSA.

FoldRx is also conducting an ongoing natural history study in patients with FAC and SSA. Little data exists on the progression of disease in patients diagnosed with FAC and SSA and the natural history study will further the characterization of the disease and its progression in patients with these diagnoses through a variety of longitudinal cardiac monitoring techniques and measurements.

**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).

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