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**FOR IMMEDIATE RELEASE**

**FOLDRX ANNOUNCES RAPID PROGRESS WITH DEVELOPMENT  
PROGRAM FOR LEAD CANDIDATE**

*-- Four ongoing studies evaluating Fx-1006A across multiple indications and genetic variations --*

**Cambridge, MA, October 7, 2008** – FoldRx Pharmaceuticals, Inc. (FoldRx) today announced that enrollment is underway in an open-label Phase II clinical study with its lead drug candidate, Fx-1006A, for patients suffering from TTR Amyloid Cardiomyopathy (ATTR-CM). The company also announced progress in several other ongoing studies that are part of a larger development program evaluating its lead candidate. Fx-1006A is designed to stop the progression of TTR amyloidosis caused by the ‘misfolding’ of a protein called transthyretin (TTR) and the subsequent accumulation of amyloid fibrils in various tissues, such as the heart and peripheral nerve tissue, with resultant cardiomyopathy and neuropathy, respectively.

Enrollment is complete in a previously announced pivotal multinational Phase II/III clinical trial of Fx-1006A for TTR amyloid polyneuropathy. Patients having completed this 18-month Phase II/III pivotal study are being enrolled in a one-year open-label follow-up study with Fx-1006A. Additionally, recruitment is underway in an open-label Phase II study for ATTR polyneuropathy patients with various TTR mutations not included in our Phase II/III trial.

“The launch of this latest study constitutes our first interventional study for cardiomyopathy stemming from TTR misfolding and the fourth study with our lead drug candidate, Fx-1006A, since initiating the development program only two years ago,” noted FoldRx President and CEO Richard Labaudinière, Ph.D. “Together, these studies will serve as the pivotal efficacy and safety data supporting registration of Fx-1006A for the treatment of TTR amyloid polyneuropathy and we anticipate results from the fully enrolled pivotal multinational Phase II/III clinical for TTR amyloid polyneuropathy by July 2009.”

In ATTR-CM, a fatal, under-diagnosed disorder, amyloid fibrils are deposited in the myocardium (the muscular layer of the heart), resulting in diastolic dysfunction (reduction in the heart’s ability to relax and fill with blood) that may progress to restrictive cardiomyopathy and symptomatic heart failure. ATTR-CM is caused by either specific point mutations in the TTR gene or age-associated TTR deposition.

“TTR amyloid cardiomyopathy is emerging as a significant cause of heart failure in the elderly population and in carriers of TTR mutations,” said Mathew Maurer, M.D., associate professor of clinical medicine, Columbia University Medical Center and director of the Clinical Cardiovascular Research Laboratory for the Elderly at the Allen Pavilion of NewYork-Presbyterian Hospital, who is the principal investigator for the CUMC trial site. “The ability to halt the deposition of TTR amyloid in ATTR-CM, for which there is currently no treatment, would represent a significant clinical advance in the care of patients with heart failure. As a clinical trial site, we at Columbia have administered the first-in-the-world trial dose of this novel drug candidate and we look forward to evaluating its efficacy as a potential benefit to patients with this rare, but serious disease.”

This open-label Phase II study is evaluating the safety and efficacy of orally-administered Fx-1006A in up to 40 patients with confirmed TTR cardiac amyloidosis. Participants will undergo a one-year treatment regimen with once a day 20mg dosing of Fx-1006A. The primary and secondary endpoints will measure TTR stabilization, safety, functional assessments including New York Heart Association Classification, clinical laboratory measurements of cardiac function, as well as ECG, Echocardiogram and Cardiac MRI measurements. These endpoints were chosen based on the findings of the TRACS study (Transthyretin Cardiac Amyloid Study), an observational study conducted by FoldRx which followed 29 patients with ATTR-CM every 6 months for up to two years. TRACS evaluated the natural history of ATTR-CM, and documented the change of various cardiac assessments in this patient population.

Labaudinière also noted, “2008 has also seen significant progress in our other ongoing programs such as our relationship with Cystic Fibrosis Foundation Therapeutics where we reached a key development milestone, discovering a series of novel drug prototypes that have shown potential *in vitro* to correct the protein-folding defect associated with cystic fibrosis.”

### **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 patients with TTR amyloidosis. 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 study 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 TTR amyloid polyneuropathy.

### **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 or due to the aging process, TTR is destabilized and misfolds, resulting in amyloid deposits in various tissues. TTR

misfolding is associated with TTR amyloidosis, occurring in patients aged 30 and above. Stabilization of transthyretin by Fx-1006A should inhibit further amyloid deposition and stop progression of ATTR, including the neuropathy and cardiomyopathy.

In patients with ATTR-CM, TTR amyloid fibrils infiltrate the myocardium of the heart, leading to diastolic dysfunction progressing to restrictive cardiomyopathy and heart failure. The predominant mutation in ATTR-CM, 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 amyloid cardiomyopathy. In the elderly, wild-type (normal) transthyretin may become structurally unstable resulting ultimately in the formation of amyloid fibrils, primarily in heart tissues. There are currently no treatments available for ATTR-CM.

In patients with ATTR-PN, deposition of TTR amyloid occurs in the peripheral nerve tissue and results in a length dependent sensorimotor neuropathy (symptoms starting in the lower extremities) and autonomic neuropathy. Liver transplantation is currently the only treatment available for these patients.

#### **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 clinical development to treat genetic neurologic and cardiovascular disorders, TTR Amyloid Polyneuropathy and TTR Amyloid Cardiomyopathy, and discovery programs in Cystic Fibrosis and neurodegenerative diseases, including 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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