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FOR IMMEDIATE RELEASE

FoldRx Pharmaceuticals Announces Positive Results from Pivotal Phase II/III Clinical Study of Tafamidis

– Tafamidis significantly halts disease progression and reduces burden of disease in patients with TTR amyloid polyneuropathy –

Cambridge, MA, July 21, 2009 – FoldRx Pharmaceuticals, Inc. (FoldRx) today announced positive results from its pivotal Phase II/III clinical study of the company's lead compound, tafamidis (Fx-1006A), in patients suffering from TTR amyloid polyneuropathy (ATTR-PN), a fatal orphan disease also known as Familial Amyloid Polyneuropathy (FAP). Liver transplantation is the only currently available treatment option for this progressive neurodegenerative disease. Preliminary results from the first randomized controlled trial ever completed in this disease demonstrate that tafamidis treatment significantly halts disease progression in ATTR-PN, reduces the burden of disease after 18 months compared to placebo, and appears to be safe and well-tolerated.

In the evaluable population, which includes all patients who completed the study per protocol, statistical significance was achieved for both primary endpoints -- disease progression as measured by the Neuropathy Impairment Score - Lower Limb (NIS-LL), and quality of life as measured by the Norfolk Quality Of Life (QOL) ($p=0.041$ and $p=0.045$, respectively). No disease progression was observed in 60% of tafamidis patients as compared to 38% of placebo patients after 18 months treatment. In addition, there was a significant deterioration in QOL in placebo patients compared to those treated with tafamidis after 18 months.

A factor in this trial was the appropriate evaluation of treatment response in patients undergoing liver transplantation. In the intent to treat population, where liver transplant patients were treated as non-responders, there was a clinically meaningful difference between the treatment groups as measured by the NIS-LL ($p=0.068$) and Norfolk ($p=0.12$). In an alternative, prospectively defined analysis that adjusted for the impact of liver transplantation, statistical significance was achieved ($p=0.039$).

In addition, a number of secondary endpoints, including objective measures of disease severity and nerve function corroborate the treatment effect seen in the primary endpoints, with tafamidis treatment resulting in statistically significant and clinically meaningful improvements when compared with placebo.

“The consistency of the results across the various endpoints and analyses clearly demonstrate the robust effect of tafamidis in halting disease progression in ATTR-PN,” noted Richard Labaudinière, Ph.D., President and CEO of FoldRx. “If approved, tafamidis would be the first disease modifying agent targeting protein misfolding. We are very excited by the results of the trial and look forward to bringing this innovative therapy to patients worldwide. We plan discussions with the U.S. and European regulatory agencies later this year and we anticipate filing marketing applications in 2010.”

In patients with ATTR-PN, amyloid fibrils, caused by the “misfolding” of a protein called transthyretin (TTR), are deposited in peripheral nerve tissues that serve limbs and organs. Tafamidis has been shown to act as a pharmacological chaperone for TTR, preventing the misfolding of the TTR protein, thus preventing the further accumulation of the amyloid fibrils on nerve tissue and protecting the patient from loss of nerve function.

“These results demonstrate that tafamidis positively alters the neurological deterioration characteristic of this disease and was well-tolerated,” said Teresa Coelho, M.D., Hospital Santo Antonio in Porto, Portugal, a principal investigator in the study and one of the worldwide experts on the disease. “ATTR-PN is a slowly progressive neurodegenerative disease that causes loss of sensation, muscle weakness and autonomic nerve dysfunction, ultimately leading to death. Tafamidis has the promise of being the first disease-modifying pharmacological therapy for ATTR-PN and offers new hope for patients suffering from this life threatening disease. Physicians will welcome this once-daily, oral treatment as an alternative to liver transplantation.”

The international, randomized, double-blind, placebo controlled pivotal study enrolled 128 patients in eight sites suffering from TTR amyloidosis polyneuropathy, with confirmed V30M TTR mutation, the most prevalent disease variant. Participants underwent an 18-month treatment regimen with once-a-day dosing of tafamidis or placebo. The co-primary endpoints measured 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. These two endpoints correlate with disease severity and are sensitive and relevant endpoints in demonstrating neuropathy disease progression.

About tafamidis

Tafamidis, the active ingredient in tafamidis meglumine (Fx-1006A) is an oral, small molecule, first-in-class pharmacological chaperone and disease modifying agent for the treatment of ATTR-PN. The drug stabilizes wild-type and variant TTR, prevents misfolding of the protein by preventing tetramer dissociation and inhibits the formation of TTR amyloid fibrils. In a dose escalating Phase I study in healthy volunteers, tafamidis was found to be safe and well-tolerated. In the pivotal clinical trial, inhibition of TTR tetramer dissociation was demonstrated in patients receiving tafamidis. This inhibition was correlated with the clinical effects of halting disease progression as evidenced by the clinical and neurophysiological endpoints.

In addition to the pivotal Phase II/III study in V30M ATTR-PN patients, an open-label Phase II study in patients with non-V30M TTR amyloidosis is ongoing. Data are expected in Q1 2010. FoldRx is also conducting a Phase II trial with tafamidis in patients with TTR amyloid cardiomyopathy, with the results anticipated in Q2 2010.

Tafamidis 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 ATTR-PN, as well as an orphan drug grant from the FDA.

Tafamidis was developed based on the pioneering work of Dr. Jeffery Kelly from Scripps Research Institute, La Jolla, CA, a scientific co-founder of FoldRx.

About Transthyretin Amyloidosis

TTR amyloidosis is a slowly progressive, multifaceted disease that primarily affects the peripheral and autonomic nervous systems and the heart. ATTR-PN is a relatively late onset autosomal dominant disease characterized by inexorable neurodegeneration associated with sensory loss, motor weakness and autonomic dysfunction, including dizziness, gastrointestinal disorders, sexual dysfunction and urinary incontinence. Epidemiology studies estimate the patient population at 10,000 worldwide.

The cardiomyopathy associated with this disease is due to infiltration of amyloid in the myocardium interstitium, leading to diastolic dysfunction progressing to restrictive cardiomyopathy and heart failure. The predominant mutation, V122I, is present in approximately four percent of the U.S. African American population. Wild-type (normal) TTR can also form amyloid, particularly in the elderly of whom approximately 15-25% of individuals over 80 have demonstrable cardiac deposition. TTR amyloidosis is ultimately fatal, with liver and/or heart transplantation as the only currently available treatments.

TTR, a transport protein for thyroxine and retinol binding protein, is produced primarily in the liver and circulates in the plasma as a tetramer. It is believed that the disease is caused by TTR tetramer dissociation into monomers that misfold resulting in amyloid deposits in various tissues. Inhibition of tetrameric dissociation, the rate limiting step in amyloid formation, should inhibit further amyloid deposition and stop progression of 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 pipeline includes a program in advanced clinical

development to treat genetic neurologic and cardiovascular disorders, Transthyretin (TTR) Amyloid Polyneuropathy (ATTR-PN) and TTR Amyloid Cardiomyopathy (ATTR-CM), and a discovery program in Parkinson's disease and cystic fibrosis, 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.