Reata Announces the Initiation of Phase 2 Studies Examining RTA 408 for the Treatment of Friedreich's Ataxia and Mitochondrial Myopathies

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IRVING, Texas, September 30, 2014 – Reata has received clearance from the Division of Neurology Products of the FDA to begin two new Phase 2 clinical programs in patients with Friedreich's Ataxia and Mitochondrial Myopathies. Both of these orphan diseases are associated with reduced energy production, fatigue, and impaired exercise capacity. There are no existing therapies specifically approved to treat patients with these diseases.

Friedreich's ataxia (FA) is an inherited disorder caused by defects in the gene for frataxin, a protein that regulates iron levels in the mitochondria. Defects in frataxin result in mitochondrial iron overload, causing impaired metabolism, oxidative stress, and damage to mitochondrial DNA. Patients with FA suffer progressive degeneration of the central and peripheral nervous systems, impaired coordination and gait, and fatigue from energy deprivation and muscle loss.

Mitochondrial Myopathies are a collection of individual orphan diseases that are associated with mitochondrial DNA mutations. These defects cause respiratory chain deficits and impaired energy production. Most of these patients share a similar phenotype characterized by skeletal muscle weakness and fatigue. These patients also may have other symptoms due to impaired energy production in other organ systems.

RTA 408 works by inducing Nrf2, which regulates multiple genes that play both direct and indirect roles in the production of cellular energy (*i.e.*, adenosine triphosphate or ATP) within the mitochondria. Directly, activation of the Nrf2 pathway increases the efficient use of fuel (fatty acids and glucose) by mitochondria and increases mitochondrial biogenesis and basal oxygen consumption. Indirectly, activation of Nrf2, through its antioxidative effects, balances reducing equivalents and maintains mitochondrial homeostasis and efficiency. In addition to its positive effects on metabolic efficiency, Nrf2 activation has been shown in preclinical studies to promote muscle repair and recovery and reduce markers of oxidative stress and muscle injury.

"Our collaborators and we have shown in preclinical studies that genetic or pharmacologic Nrf2 activation positively regulates mitochondrial function and energy production. We hope to translate this effect into improved physical functioning and reduced fatigue in patients with Friedreich's ataxia and mitochondrial myopathies. These rare, debilitating diseases currently have no approved therapies," noted Dr. Colin Meyer, Reata's Chief Medical Officer.

The two initial Phase 2 trials will both be multi-center, double-blind, randomized, dose-ranging, placebo-controlled studies. The primary efficacy endpoint in both studies will be peak work as assessed during exercise testing. The studies will also explore changes in other measures of physical activity, fatigue, and biomarkers associated with mitochondrial functioning.

About Reata Pharmaceuticals, Inc.

Reata Pharmaceuticals, Inc. is a privately held company aiming to translate innovative research into breakthrough medicines for difficult diseases that have significant unmet needs. Reata is the leader in developing a novel class of drugs with potent transcription-regulating activity called antioxidant inflammation modulators (AIMs). AIMs activate Nrf2, promoting the production of numerous antioxidant, detoxification, and anti-inflammatory genes, and inhibit NF-κB, a transcription factor that regulates many pro-inflammatory proteins. The pharmacology of the AIMs mimics that of endogenous prostaglandin metabolites that are responsible for the orchestrated resolution of inflammation. The anti-inflammatory, cytoprotective and energy metabolism effects of AIM pharmacology have been documented in more than 250 scientific papers and are potentially relevant to a wide range of diseases.

Due to the broad applicability of AIM biology, Reata is actively conducting or initiating phase 2 programs with AIMs in multiple therapeutic areas, including pulmonary arterial hypertension, oncology, Friedreich's ataxia, mitochondrial myopathies, dermatology, and ophthalmology.

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