Retrophin to Highlight Fosmetpantotenate Program for PKAN at Upcoming Medical Congresses

October 3, 2018

SAN DIEGO, Oct. 03, 2018 (GLOBE NEWSWIRE) -- Retrophin, Inc. (NASDAQ: RTRX) today announced that presentations highlighting the design of the ongoing pivotal Phase 3 FORT Study of fosmetpantotenate for the treatment of pantothenate kinase-associated neurodegeneration (PKAN), the patient and caregiver experience with PKAN, and the diagnostic pathway and clinical experience of patients with PKAN, will be presented by the Company and its collaborators at upcoming medical congresses in October.

Abstract Title: The Fosmetpantotenate Replacement Therapy (FORT) Pivotal Trial: Utilization of a Novel Primary Efficacy Outcome in Patients with Pantothenate Kinase-Associated Neurodegeneration

Meeting: International Congress of Parkinson's Disease and Movement Disorders ® (MDS), taking place from October 5-9, 2018, in Hong Kong.
Abstract Program #: 25
Poster Session: Clinical Trials and Therapy in Movement Disorders
Location: Hall 3FG, Hong Kong Convention and Exhibition Centre
*Date & Time:*Saturday, October 6, 2018, 1:45 p.m. – 3:15 p.m. HKT

Meeting: 47th Annual Child Neurology Society (CNS) Annual Meeting, taking place from October 15-18, 2018, in Chicago, IL.
Poster #: 104
Location: Riverside Exhibit Hall (East Tower), Hyatt Regency Chicago
*Date & Time:*Tuesday, October 16, 2018, 12:45 p.m. – 2:00 p.m. & 4:00 p.m. – 5:30 p.m. CT & Wednesday, October 17, 2018, 7:00 a.m. – 8:15 a.m. CT

***Abstract Title:*Patient and Caregiver Experience With Pantothenate Kinase-Associated Neurodegeneration**

Meeting: International Congress of Parkinson's Disease and Movement Disorders ® (MDS), taking place from October 5-9, 2018, in Hong Kong.
Abstract Program #: 484
Poster Session: Rare Genetic and Metabolic Diseases
Location: Hall 3FG, Hong Kong Convention and Exhibition Centre
*Date & Time:*Saturday, October 6, 2018, 1:45 p.m. – 3:15 p.m. HKT

Meeting: 47th Annual Child Neurology Society (CNS) Annual Meeting, taking place from October 15-18, 2018, in Chicago, IL.
Poster #: 103
Location: Riverside Exhibit Hall (East Tower), Hyatt Regency Chicago
*Date & Time:*Tuesday, October 16, 2018, 12:45 p.m. – 2:00 p.m. & 4:00 p.m. – 5:30 p.m. CT & Wednesday, October 17, 2018, 7:00 a.m. – 8:15 a.m. CT

***Abstract Title:*Diagnostic Pathway and Clinical Experience of Patients With High Versus Low Severity Pantothenate Kinase-Associated Neurodegeneration (PKAN)**

Meeting: 143rd Annual Meeting of the American Neurological Association (ANA), taking place from October 21-23, 2018, in Atlanta, GA.
Abstract Program #: S224
Poster Session: P1
*Location:*Grand Hall, Hyatt Regency Atlanta
*Date & Time:*Sunday, October 21, 2018, 5:30 p.m. – 7:00 p.m. ET

About Fosmetpantotenate

Fosmetpantotenate is a novel investigational small molecule replacement therapy designed to pass the blood-brain barrier and be converted to phosphopantothenic acid (PPA). PPA synthesis is a key step in the biosynthesis of CoA, which is essential in biochemical reactions impacting energy metabolism, membrane integrity, signaling and other critical processes. Preclinical findings suggest fosmetpantotenate has the ability to pass the blood-brain barrier and restore CoA levels.

Fosmetpantotenate, which has the potential to be the first approved treatment targeting the underlying cause of PKAN, has been granted orphan drug designation for the treatment of PKAN by the U.S. Food and Drug Administration (FDA) and European Commission, as well as Fast Track status in the U.S. by the FDA. In a Phase 1 study, fosmetpantotenate was found to be generally well-tolerated in healthy volunteers and it is currently being evaluated in the pivotal Phase 3 FORT Study conducted under a Special Protocol Assessment (SPA) agreement with the FDA. The Company anticipates completion of patient enrollment in the FORT Study around year-end 2018, and top-line data to become available in the second half of 2019.

About Pantothenate Kinase-Associated Neurodegeneration

PKAN is a rare, genetic and life-threatening neurological disorder characterized by a host of progressively debilitating symptoms that typically begin in early childhood. People suffering from PKAN may experience movement disorders such as dystonia (sustained muscle contraction leading to abnormal posture), rigidity, dysphagia (problems swallowing), twisting and writhing, as well as visual impairment. PKAN is estimated to affect up to 5,000 people worldwide.

PKAN is caused by a mutation in the *PANK2* gene, which encodes a critical protein that phosphorylates vitamin B5 (pantothenate), generating phosphopantothenate. The disruption of this metabolic pathway ultimately leads to decreased levels of CoA.

About Retrophin

Retrophin is a biopharmaceutical company specializing in identifying, developing and delivering life-changing therapies to people living with rare disease. The Company's approach centers on its pipeline featuring late-stage assets targeting rare diseases with significant unmet medical needs, including fosmetpantotenate for pantothenate kinase-associated neurodegeneration (PKAN), a life-threatening neurological disorder that typically begins in early childhood, and sparsentan for focal segmental glomerulosclerosis (FSGS) and IgA nephropathy (IgAN), disorders characterized by progressive scarring of the kidney often leading to end-stage renal disease. Research in additional rare diseases is also underway, including a joint development arrangement evaluating the potential of CNSA-001 in phenylketonuria (PKU), a rare genetic metabolic condition that can lead to neurological and behavioral impairment. Retrophin's R&D efforts are supported by revenues from the Company's commercial products Chenodal ®, Cholbam® and Thiola®.

Retrophin.com

Forward-Looking Statements

This press release contains "forward-looking statements" as that term is defined in the Private Securities Litigation Reform Act of 1995. Without limiting the foregoing, these statements are often identified by the words "may", "might", "believes", "thinks", "anticipates", "plans", "expects", "intends" or similar expressions. In addition, expressions of our strategies, intentions or plans are also forward-looking statements. Such forward-looking statements are based on current expectations and involve inherent risks and uncertainties, including factors that could delay, divert or change any of them, and could cause actual outcomes and results to differ materially from current expectations. No forward-looking statement can be guaranteed. Among the factors that could cause actual results to differ materially from those indicated in the forward-looking statements are risks and uncertainties associated with the Company's business and finances in general, success of its commercial products as well as risks and uncertainties associated with the Company's preclinical and clinical stage pipeline. Specifically, with respect to fosmetpantotenate, the Company faces risk that the Phase 3 clinical trial of fosmetpantotenate will not demonstrate that fosmetpantotenate is safe or effective or serve as the basis for an NDA filing as planned; risk that fosmetpantotenate will not be approved for efficacy, safety, regulatory or other reasons, risk associated with enrollment of clinical trials for rare diseases and risk the clinical trial may not succeed or may be delayed for safety, regulatory or other reasons. The Company faces risk that it will be unable to raise additional funding that may be required to complete development of any or all of its product candidates; risk relating to the Company's dependence on contractors for clinical drug supply and commercial manufacturing; uncertainties relating to patent protection and intellectual property rights of third parties; and risks and uncertainties relating to competitive products and technological changes that may limit demand for the Company's products. You are cautioned not to place undue reliance on these forward-looking statements as there are important factors that could cause actual results to differ materially from those in forward-looking statements, many of which are beyond our control. The Company undertakes no obligation to publicly update any forward-looking statement, whether as a result of new information, future events or otherwise. Investors are referred to the full discussion of risks and uncertainties as included in the Company's most recent Form 10-K, Form 10-Q and other filings with the Securities and Exchange Commission.

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