Retrophin Unveils New Clinical Development Candidate RE-034, An ACTH Analog

December 17, 2013

Company to Initiate Clinical Development in Multiple Indications

Management to Host Conference Call and Webcast Tomorrow at 9:00 a.m. ET

NEW YORK--(BUSINESS WIRE)-- Retrophin, Inc. (OTCQB: RTRX) today announced its newest clinical development candidate RE-034 (cosyntropin), a long-acting synthetic analog (amino acids 1-24) of the naturally-occurring adrenocorticotropic hormone (ACTH) formulated with zinc. Cosyntropin is also known outside the U.S. as tetracosactide.

Retrophin plans to file an Investigational New Drug (IND) Application and initiate a Phase 1 pharmacokinetic trial of RE-034 in the first half of 2014. Shortly thereafter, the company plans to initiate Phase 3 pivotal clinical trials of RE-034 for the treatment of infantile spasms, or West Syndrome, a catastrophic form of epilepsy, and nephrotic syndrome, a devastating condition marked by very high levels of proteinuria. Commercially available ACTH formulations that are substantially similar to RE-034 have been shown to be efficacious in treating these diseases. Retrophin recently met with the U.S. Food and Drug Administration (FDA) and agreed with the FDA on a clinical trial design for approval in West Syndrome.

"Melanocortin agonists have shown profound activity in nephrology and neurology," said Martin Shkreli, Founder and Chief Executive Officer of Retrophin. "These are two key therapeutic areas for Retrophin, so approximately a year ago we embarked on development of RE-034 and have recently reached major CMC milestones. Our planned robust clinical trials will add to the understanding of this class of compounds, and we look forward to exploring RE-034 as a treatment for additional grave diseases. Furthermore, our synthetic ACTH can potentially bring relief to the high cost burden associated with the currently available animal-derived formulation in the U.S."

"This is promising news for patients who suffer from nephrotic syndrome and infantile spasms, two very serious conditions," said Horacio Plotkin, M.D., Chief Medical Officer of Retrophin. "We expect that physicians would welcome the potential benefits and consistency of our compound, as synthetic medicines are often preferable to animal-derived drugs."

Conference Call Information

Retrophin will host a conference call and webcast (with slides) tomorrow, Wednesday, Dec. 18, 2013, at 9:00 a.m. ET to discuss development plans for RE-034. To participate in the conference call, dial 1-800-591-6942 (U.S.) or 1-617-614-4909 (International), confirmation code 38074071, shortly before 9:00 a.m. tomorrow. The audio webcast and slides can be accessed at www.retrophin.com, in the Events and Presentations section. A replay of the call will be available from Wednesday, Dec. 18, 11:00 a.m. ET to Wednesday, Dec. 25, 11:59 p.m. ET. The replay number is 1-888-286-8010 (U.S.) or 1-617-801-6888 (International), confirmation code 18030776.

About Retrophin

Retrophin is a pharmaceutical company focused on the development, acquisition and commercialization of drugs for the treatment of serious, catastrophic or rare diseases for which there are currently no viable options for patients. The Company's pipeline includes compounds for several catastrophic diseases, including Focal Segmental Glomerulosclerosis (FSGS), Pantothenate Kinase-Associated Neurodegeneration (PKAN), Duchenne Muscular Dystrophy and others. Retrophin's lead compound, sparsentan, also known as RE-021, is scheduled to begin enrollment in a potentially pivotal Phase 2 clinical trial for FSGS during 2013. The Company intends to reintroduce Syntocinon Nasal Spray to assist initial postpartum milk ejection and also initiate clinical trials for the drug as a potential treatment for schizophrenia and autism. Retrophin plans to initiate a Phase 1 pharmacokinetic trial of RE-034, an ACTH analog, in the first half of 2014. For additional information, please visit www.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, regarding the research, development and commercialization of pharmaceutical products. 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. Forward-looking statements in the press release should be evaluated together with the many uncertainties that affect the Company's business. 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 filings with the Securities and Exchange Commission.

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