Retrophin Signs U.S. License Agreement for Syntocinon™ Nasal Spray (Oxytocin)

December 12, 2013

Retrophin to reintroduce Syntocinon™ Nasal Spray in the U.S. and Develop the Drug for Schizophrenia and Autism

Management to Host Conference Call and Webcast Today at 5:30 p.m. ET

NEW YORK--(BUSINESS WIRE)-- Retrophin, Inc. (OTCQB:RTRX) today announced that it has signed an agreement with Novartis Pharma AG for an exclusive U.S. license for Syntocinon™ Nasal Spray, the intranasal formulation of a synthetic version of the naturally occurring peptide hormone oxytocin, for an upfront payment of $5.0 million plus milestone payments and royalties.

Syntocinon™ Nasal Spray was approved in the U.S. in 1960 to assist initial postpartum milk ejection, but was discontinued by Novartis in 1997 for commercial reasons. Retrophin plans to reintroduce the product for prescription use in the U.S. in Q2 2014. Additionally, the company intends to pursue a clinical trial program with Syntocinon™ for its potential use as a treatment for schizophrenia and autism.

Difficulty breastfeeding newborn infants is an unmet medical need. There is no FDA-approved drug currently available in the U.S. for this patient population. In a Retrophin-conducted survey of 50 obstetricians, respondents indicated that 16% of new mothers in their care have problems with milk let-down, and 79% of these physicians said they would use Syntocinon™ if it became available for this indication.

One percent of Americans suffer from schizophrenia according to National Institute of Mental Health estimates. Over the past four years, three randomized, double-blind, placebo-controlled, independent proof-of-concept schizophrenia trials were conducted in patients whose symptoms were inadequately controlled despite receiving therapeutic doses of an atypical antipsychotic[1,2,3]. All three studies suggest that intranasal oxytocin administered as an adjunct to subjects' antipsychotic drugs improves positive and negative symptoms, as assessed by the Positive and Negative Syndrome Scale (PANSS), significantly more than placebo.

Preclinical evidence indicates that oxytocin has a critical role in the regulation of brain-mediated processes that are strongly relevant to many neuropsychiatric disorders. Small studies suggest that even a single dose of intranasally delivered oxytocin can have significant, pro-social effects on human behavior. Finally, there have been several positive findings with oxytocin in animal models of schizophrenia.

Commenting on today's news, Martin Shkreli, Founder and Chief Executive Officer of Retrophin, noted, "We believe the reintroduction of intranasal Syntocinon™ for lactation deficiency would be a welcome option for obstetricians and their patients, while providing Retrophin with a revenue stream. Equally important, we believe that Syntocinon™ is an underutilized drug. Compelling data from studies that show positive results in patients with schizophrenia and autism lead us to believe that this drug may have significant utility in treating these conditions. We look forward to initiating a clinical program in order to develop the drug for these indications."

Clinical studies also suggest that oxytocin may have positive effects in patients with autism spectrum disorders. Studies have shown that the administration of oxytocin improves mental representations of self, social cognition, and quality of life in patients with autism. Evidence suggests that, under oxytocin, patients respond more strongly to others and exhibit more appropriate social behavior and affect, suggesting a therapeutic potential of oxytocin through its action on a core dimension of autism. Large, controlled studies are warranted to confirm these findings.

In a recent article discussing oxytocin as a potential therapeutic target for schizophrenia and other neuropsychiatric conditions, Dr. David Feifel (Department of Psychiatry, University of California, San Diego, San Diego, CA) stated that if preliminary results are confirmed in larger trials, "…it may signal the beginning of an exciting new era in the treatment of schizophrenia and perhaps other neuropsychiatric disorders, something desperately needed given the disappointing lack of progress in developing efficacious novel mechanism treatments in this field."[4]

Conference Call Information

Retrophin will host a conference call and webcast (with slides) today, Thursday, December 12, at 5:30 p.m. to discuss development and commercial plans for Syntocinon™ Nasal Spray. To participate in the conference call, dial 1-877-299-4454 (U.S.) or 1-617-597-5447 (International), confirmation code 84581984 shortly before 5:30 p.m. ET. 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 9:30 p.m. ET on Thursday, December 12, through 11:59 p.m. ET on Thursday, December 19. The replay number is 1-888-286-8010 (U.S.) or 1-617-801-6888 (International), confirmation code 76349669.

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, formerly known as RE-021, is scheduled to begin enrollment in a potentially pivotal Phase 2 clinical trial for FSGS during 2013. The Company also intends to initiate clinical trials for intranasal oxytocin as a potential treatment for schizophrenia and autism. 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.

[1] Feifel et al., Biological Psychiatry (2010) 68:678-680

[2] Pedersen et al., Schizophrenia Research 132 (2011) 50-53

[3] Modabbernia et al., CNS Drugs (2013) 27:57-65

[4] Neuropsychopharmacology Reviews (2012) 37, 304—305

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Source: Retrophin