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Ventrus Biosciences Announces Positive Results From Clinical Dermal Safety and Pharmacokinetic Studies of Diltiazem (VEN 307)

Results From Second Pivotal Phase 3 Trial in Anal Fissures Expected First Quarter 2014

NEW YORK, Sept. 11, 2013 (GLOBE NEWSWIRE) -- Ventrus Biosciences, Inc. (Nasdaq:VTUS), a pharmaceutical company focused on developing and commercializing gastrointestinal products, today announced positive results from two clinical dermal safety studies and one pharmacokinetic (PK) study of diltiazem hydrochloride 2% cream (VEN 307). All three studies were conducted to support the Company's planned New Drug Application (NDA) for VEN 307 as a treatment for anal fissures (AF).

For the dermal safety studies, Ventrus conducted two single-center, randomized, controlled trials to evaluate the irritation and sensitization potential of VEN 307 in healthy volunteers. The studies utilized cumulative as well as repeat insult patch designs, which aim to provide a standard assessment of cutaneous tolerability and safety. In these studies, results demonstrated that VEN 307 was safe and well tolerated. Irritation and sensitization caused by VEN 307 was similar to that seen with both placebo and saline, and was significantly better than that seen with sodium lauryl sulfate (SLS), the positive control. Minimal adverse events (AE) and no severe or serious AEs were reported.

The Company also announced results from a pharmacokinetic (PK) study comparing VEN 307 to oral diltiazem in subjects with anal fissure. All PK parameters, including AUC, Cmax, Tmax and half-life, were consistent with expectations, and results demonstrated that systemic exposure of VEN 307 was approximately only 10% that of oral diltiazem, in line with prior data from an investigator sponsored trial with this product, and confirming a potentially high safety margin.

"These results mark another important step in our effort to develop and commercialize VEN 307 as a treatment for anal fissures," said Russell H. Ellison, M.D., M.Sc., Chairman and Chief Executive Officer of Ventrus Biosciences, Inc. "With enrollment in our second, pivotal Phase 3 study moving toward completion, we remain focused on conducting a high-quality trial with the appropriate patient population and rigorous inclusion/exclusion criteria. We have added several new clinical sites in Europe to help expedite screening and enrollment, and now expect to complete enrollment near the end of this year, with data expected in the first quarter of 2014. Based on our anticipated timeline, we believe we remain sufficiently capitalized to support operations through a potential launch of VEN 307."

VEN 307 is currently being studied in a second pivotal trial, a Phase 3b, randomized, double-blind, placebo-controlled, parallel-treatment group, multicenter efficacy and safety study in subjects with AF (VEN307-AF-001). The study is expected to enroll 400 subjects at approximately 140 clinical sites in the U.S., Europe, Canada, and Israel. The primary objective is to evaluate the efficacy of VEN 307 on reduction of worst AF-related pain associated with or following defecation when administered three times a day for 28 days. The secondary objectives are to evaluate the effect of VEN 307 on reduction of overall daily AF-related pain and to evaluate patient global impression of improvement (PGI-I) at Day 29 in subjects with AF-related pain.

Results from this ongoing pivotal Phase 3 study of VEN 307 are expected in the first quarter of 2014 and, assuming a successful outcome, Ventrus expects to file an NDA in the second quarter of 2014.

Ventrus reported positive results in 2012 from its first pivotal Phase 3, randomized, double-blind, placebo-controlled clinical trial of VEN 307 for the treatment of AF. The trial randomized 465 subjects to diltiazem hydrochloride 4% or 2% w/w cream, or placebo, applied topically three times daily (TID) for 8 weeks, followed by a 4 week blinded observation period. At 4 weeks, the 2% diltiazem treatment arms demonstrated improvements compared to placebo in the primary endpoint of average of worst anal pain associated with or following defecation (pain score improvement of 0.43, p=0.0122) and in the secondary endpoints of overall anal-fissure-related pain (pain score of 0.42, p=0.0143). Pain endpoints were assessed using an 11-point numerical pain rating scale (Likert-like scale).

Because diltiazem is approved in oral formulations for the treatment of angina and high blood pressure, VEN 307 is eligible for the FDA's 505(b)2 registration pathway.

Dermal Safety and PK Study Design Details

The clinical dermal irritation study was conducted in 30 subjects using 0.2 g of diltiazem hydrochloride 2% cream, 0.2 g placebo cream, 0.2 mL of solution of 0.2% SLS as positive control, and 0.2 mL of 0.9% saline as negative control applied topically under occlusive patch conditions to the infrascapular area of the back, once daily for 21 consecutive days. AE data was collected

throughout the duration of the study.

The clinical dermal sensitization study was conducted in 200 subjects using 0.2 g of diltiazem hydrochloride 2% cream, 0.2 g placebo cream, 0.2 mL of solution of 0.1% SLS as positive control, and 0.2 mL of 0.9% saline as negative control applied topically 3 times weekly for 21 days (9 applications) during the Induction Phase, and one time at Challenge (10 times in total). AE data was collected throughout the duration of the study.

The clinical PK study was an open-label, single- and multi-dose study comparing VEN 307 to single-dose oral diltiazem in subjects with anal fissure. Twelve subjects were enrolled in this study which evaluated AUC, Cmax, Tmax, and half-life.

About Anal Fissures

Anal fissure is a tear in the lining of the anal canal characterized by severe anal pain associated with or after bowel movements. It is a common anal disorder, which we believe is underdiagnosed. The pathogenesis of anal fissure is hypothesized to be initiated by the passage of a hard fecal bolus, resulting in a split in the epithelium of the anal canal. Along with poor vascular supply of the anal epithelium, increased activity (tone) of the internal anal sphincter smooth muscle further compromises the anodermal blood supply and contributes to the pain and ischemia of the anal epithelium, perpetuating ulceration and preventing healing.

In 2010, it was estimated by SDI Health LLC that there were approximately 1.1 million office visits per year for anal fissures. Topical diltiazem, which is not approved by the FDA as a use for anal fissure, is currently listed in the U.S. anal fissure treatment guidelines as a preferred agent prior to attempting surgery, and is available only as a compounded medicine.

About VEN 307: Diltiazem Hydrochloride cream

Diltiazem hydrochloride is a calcium-channel blocker that has been marketed in oral formulations for the treatment of angina and high blood pressure for over two decades. Diltiazem hydrochloride cream is applied perianally to treat pain related to anal fissure. It has been shown to normalize internal anal sphincter pressure and reduce anal maximal resting pressure, or MRP, and its vasodilator activity has the potential to improve blood supply, thereby decreasing the pain associated with anal fissures.

About Ventrus

Ventrus is a development stage pharmaceutical company focused on the development of late-stage prescription drugs for gastrointestinal problems, specifically anal disorders. Our lead product is topical diltiazem (VEN 307) for the treatment of anal fissures, for which the first Phase 3 trial was initiated in November 2010, and reported positive top line results in May 2012. The second Phase 3 trial began enrollment in the fourth quarter of 2012 and is ongoing. Our product candidate portfolio also includes topical phenylephrine (VEN 308) intended to treat fecal incontinence. VEN 307 and VEN 308 are two molecules that were previously approved and marketed for other indications and that have been formulated into our in-licensed proprietary topical treatments for these new gastrointestinal indications.

Please Note: The information provided herein contains estimates and other forward-looking statements regarding future events. Such statements are just predictions and are subject to risks and uncertainties that could cause the actual events or results to differ materially. These risks and uncertainties include, among others: the components, timing, cost and results of clinical trials and other development activities involving our product candidates; the unpredictability of the clinical development of our product candidates and of the duration and results of regulatory review of those candidates by the FDA and foreign regulatory authorities; the unpredictability of the size of the markets for, and market acceptance of, any of our products; our anticipated capital expenditures, our estimates regarding our capital requirements, and our need for future capital; our reliance on our lead product candidate, VEN 307; our ability to retain and hire necessary employees and to staff our operations appropriately; and the possible impairment of, or inability to obtain, intellectual property rights and the costs of obtaining such rights from third parties. The reader is referred to the documents that we file from time to time with the Securities and Exchange Commission.

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