

## NEWS RELEASE

# Acorda Announces Results from Phase 2b Clinical Trial of CVT-301 for Treatment of OFF Periods in Parkinson's Disease Published in Movement Disorders

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ARDSLEY, N.Y.--(BUSINESS WIRE)-- Data from the Phase 2b clinical trial of CVT-301, **published this week in the peer-reviewed journal Movement Disorders**, showed that people with Parkinson's disease (PD) experiencing OFF periods and who were treated with CVT-301 had significantly greater improvements in motor function than those treated with placebo. A treatment effect was evident at 10 minutes after dosing and was sustained for at least one hour, the longest time point at which patients were assessed.

Acorda Therapeutics, Inc. (NASDAQ:ACOR) is developing CVT-301 for the treatment of OFF periods in people with PD.

"Oral levodopa is a cornerstone of Parkinson's disease treatment, but after chronic therapy, approximately half of people with PD who are treated with oral L-dopa experience OFF periods," said Peter LeWitt, M.D., M.Med.Sc., Director of the PD and Movement Disorders Program at Henry Ford Hospital and lead author of the study. "OFF periods tend to increase in frequency, severity and duration during the course of the disease, and are among the most challenging experiences for people with this disorder. Based on the data from this Phase 2b clinical study, CVT-301 has the potential to restore motor function to people with Parkinson's disease when they experience OFF periods, and this is now being studied in a Phase 3 clinical trial."

OFF periods are characterized by a re-emergence of PD symptoms, including motor symptoms such as the impaired ability to move, muscle stiffness and tremor. This re-emergence can occur even when treatment regimens of oral levodopa (L-dopa) and other standard of care medications have been optimized.

The Phase 2b trial was a randomized, double blind, placebo-controlled, multicenter study in 86 people with PD for the treatment of OFF periods. Participants were randomized to self-administer CVT-301 or placebo to the lung via an inhaler, as an adjunct to their established oral PD medications. Participants received 35mg of CVT-301 or placebo in study weeks 1 and 2, and 50mg of drug or placebo in weeks 3 and 4.

The primary endpoint was defined as the mean change from baseline OFF state in Unified Parkinson's Disease Rating Scale motor function (UPDRS Part III) score, (measured at 10-60 minutes post dose) after 4 weeks of treatment. The UPDRS is an established assessment method to monitor PD motor impairment that has been used extensively in clinical research.

In this study, participants receiving CVT-301 showed a statistically significant and clinically important reduction in average UPDRS III score compared to placebo (p<0.01) and across all measured time points beginning at 10 and up to 60 minutes post-administration (p<0.05). There were no concerning safety signals observed in either CVT-301 dose group, with no increase relative to placebo in troublesome or non-troublesome dyskinesias during ON periods. There were no serious adverse events reported in the drug group, and the incidence of drug-related adverse events was similar between treatment groups (23% drug group; 21% placebo group). The most common adverse events were dizziness (7% drug group; 5% placebo), cough (7% drug group; 2% placebo) and nausea (7% drug group; 0% placebo); there were no adverse events related to cardiovascular or lung function. PD patients were able to self-administer treatment while in an OFF state.

Earlier this week, data included in this paper were featured at the 68th Annual Meeting of the American Academy of Neurology (AAN) during the Movement Disorders Invited Science Session. Data from the Phase 2b study were also previously presented at the International Congress of Parkinson's Disease and Movement Disorders (MDS) annual meetings in 2014 and 2015. The 2015 presentation was recognized in the meeting's Blue Ribbon Highlights Session.

# About CVT-301/Phase 3 Program

CVT-301 is an investigational agent being developed as a self-administered, inhaled levodopa (L-dopa) therapy for the as-needed treatment of OFF periods in Parkinson's disease (PD). It is intended for use as an adjunctive therapy to a patient's individually optimized oral L-dopa/carbidopa regimen.

CVT-301 utilizes Acorda's ARCUS® platform for inhaled therapeutics, which delivers a precise dose of a dry powder formulation of levodopa to the lung. Oral medication can be associated with slow onset of action, as the medicine is absorbed through the gastrointestinal (digestive) tract before reaching the brain. Inhaled treatments, such as those that utilize our ARCUS technology, enter the body through the lungs and reach the brain shortly thereafter, bypassing the digestive system.

Based on the results of the Phase 2b trial, Acorda has initiated a Phase 3 clinical trial that is expected to enroll approximately 345 participants across three arms: 50mg, 35mg, or placebo. These are the same doses used in the

Phase 2b study. The primary outcome measure is improvement on the Unified Parkinson's Disease Rating Scale Part 3 (UPDRS III) after administration of CVT-301 in patients experiencing an OFF period (30 minutes post dose). UPDRS III is an established scale to monitor PD motor impairment, and is considered a standard in the field.

More details about the study, including enrollment criteria, can be found

at https://cvt301.acordatrials.com/en/patient/ or http://clinicaltrials.gov/ct2/show/NCT02240030?term=CVT-301&rank=2.

# **About Acorda Therapeutics**

Founded in 1995, Acorda Therapeutics is a biotechnology company focused on developing therapies that restore function and improve the lives of people with neurological disorders.

Acorda has an industry leading pipeline of novel neurological therapies addressing a range of disorders, including Parkinson's disease, epilepsy, post-stroke walking deficits, migraine, and multiple sclerosis. Acorda markets three FDA-approved therapies, including **AMPYRA®** (dalfampridine) Extended Release Tablets, 10 mg.

For more information, please visit www.acorda.com.

# Forward-Looking Statement

This press release includes forward-looking statements. All statements, other than statements of historical facts, regarding management's expectations, beliefs, goals, plans or prospects should be considered forward-looking. These statements are subject to risks and uncertainties that could cause actual results to differ materially, including: the ability to complete the Biotie transaction on a timely basis or at all; the ability to realize the benefits anticipated from the Biotie and Civitas transactions, among other reasons because acquired development programs are generally subject to all the risks inherent in the drug development process and our knowledge of the risks specifically relevant to acquired programs generally improves over time; the ability to successfully integrate Biotie's operations and Civitas' operations, respectively, into our operations; we may need to raise additional funds to finance our expanded operations and may not be able to do so on acceptable terms; our ability to successfully market and sell Ampyra in the U.S.; third party payers (including governmental agencies) may not reimburse for the use of Ampyra or our other products at acceptable rates or at all and may impose restrictive prior authorization requirements that limit or block prescriptions; the risk of unfavorable results from future studies of Ampyra or from our other research and development programs, including CVT-301, Plumiaz (diazepam) Nasal Spray, or any other acquired or in-licensed programs; we may not be able to complete development of, obtain regulatory approval for, or successfully market CVT-301, Plumiaz, any other products under development, or the products that we would acquire if we complete the Biotie transaction; the occurrence of adverse safety events with our products; delays in obtaining or failure to obtain and maintain regulatory approval of or to successfully market Fampyra outside of the U.S. and our dependence on our collaborator Biogen in connection therewith; competition; failure to protect our

intellectual property, to defend against the intellectual property claims of others or to obtain third party intellectual property licenses needed for the commercialization of our products; and failure to comply with regulatory requirements could result in adverse action by regulatory agencies.

These and other risks are described in greater detail in our filings with the Securities and Exchange Commission. We may not actually achieve the goals or plans described in our forward-looking statements, and investors should not place undue reliance on these statements. Forward-looking statements made in this release are made only as of the date hereof, and we disclaim any intent or obligation to update any forward-looking statements as a result of developments occurring after the date of this release.

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