NEWS RELEASE

# Data from Clinical and Preclinical Trials of CVT-301 for Treatment of OFF Periods in Parkinson's Disease Published in Science Translational Medicine

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ARDSLEY, N.Y.--(BUSINESS WIRE)-- Results from Phase 1, Phase 2a and preclinical studies of CVT-301, an inhaled form of levodopa, have been featured in the current edition of **Science Translational Medicine**. **Acorda Therapeutics, Inc.** (NASDAQ:ACOR) is developing CVT-301 for the treatment of OFF periods in people with Parkinson's disease (PD).

As PD progresses, people with Parkinson's experience OFF periods, which are characterized by the re-emergence of PD symptoms. These include motor symptoms such as impaired movement, muscle stiffness, and tremor, as well as non-motor symptoms. This re-emergence can occur even when an individual's treatment regimen has been optimized. OFF periods typically increase in frequency during the course of the disease.

"OFF periods can be hugely disruptive to the lives of people with Parkinson's and their families, and are considered one of the greatest unmet medical needs in the treatment of Parkinson's," said Ron Cohen, M.D., President and CEO of Acorda Therapeutics. "CVT-301 is an inhaled powder form of levodopa that is being studied in combination with standard of care Parkinson's disease regimens. Following two successful Phase 2 clinical trials1 ,2, our Phase 3 program is assessing the extent to which CVT-301, used when people with Parkinson's begin to experience OFF periods, can restore motor function. If approved, CVT-301 may provide a valuable new treatment option for these individuals."

Acorda's Phase 3 clinical program comprises a Phase 3 safety and efficacy study as well as general and special population safety studies. The program is designed to confirm the efficacy and safety profile of CVT-301 and support global regulatory marketing authorization applications. The Company expects to announce results from its randomized, placebo-controlled Phase 3 trial in Q1 2017.

## About Parkinson's Disease and OFF Periods

There are approximately one million people in the U.S. and 1.2 million people in Europe diagnosed with Parkinson's disease (PD). PD is a progressive neurodegenerative disorder resulting from the gradual loss of certain neurons responsible for producing dopamine, which causes impairment of motor function including impaired movement, muscle stiffness and tremors. Non-motor symptoms are also common; they include anxiety, depression, sleep difficulties and gastrointestinal (GI) disorders among others.

In the United States, approximately 350,000 people with Parkinson's experience OFF periods.

## 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 treatment of OFF periods in Parkinson's disease. 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 L-dopa to the lung. Oral medication can be associated with slow and variable 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 successful results of two Phase 2 trials, Acorda initiated a Phase 3 clinical program that includes a safety and efficacy study as well as general and special population safety studies. The efficacy trial has enrolled 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. The Company expects to announce results from this study in Q1 2017.

Early CVT-301 clinical studies were funded in part by grants from The Michael J. Fox Foundation for Parkinson's Research.

## 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 a pipeline of novel neurological therapies addressing a range of disorders, including Parkinson's disease, post-stroke walking difficulties (PSWD), migraine, and multiple sclerosis. Acorda markets three FDA-

approved therapies, including AMPYRA® (dalfampridine) Extended Release Tablets, 10 mg.

For more information, please visit the Company's website at: **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; 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 (dalfampridine) Extended Release Tablets, 10 mg 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 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, any other products under development, or the products that we will acquire when 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 presentation 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 presentation.

1 Lipp MM et al. Preclinical and clinical assessment of inhaled levodopa for OFF episodes in Parkinson's disease. Sci. Transl. Med. 8, 360ra136 (2016) [Online version].

2 LeWitt PA et al. Randomized Trial of Inhaled Levodopa (CVT-301) for Motor Fluctuations in Parkinson's Disease. Movement Disorders 2016; 31(9): 1356-1365

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Acorda Therapeutics

Jeff Macdonald, 914-326-5232

**jmacdonald@acorda.com**