CVT-301 Phase 3 Data Showed Significantly Improved Motor Function During OFF Periods in Parkinson’s Disease

6/5/2017

● CVT-301 Phase 3 SPAN-PD study met primary endpoint of improvement in UPDRS III, in data presented at 2017 International Congress of Parkinson’s Disease and Movement Disorders (MDS)

● Multiple secondary endpoints were supportive of primary endpoint result

● Acorda plans to file New Drug Application (NDA) in U.S. by end of Q2 2017

● Company to host investor webcast to review data from CVT-301 clinical program on Monday, June 5 at 4:30 pm Eastern / 1:30 pm Pacific

ARDSLEY, N.Y.--(BUSINESS WIRE)-- Acorda Therapeutics, Inc. (Nasdaq:ACOR) presented data from its Phase 3 SPAN-PD clinical trial of CVT-301 (levodopa inhalation powder) that showed a statistically significant, clinically meaningful improvement in motor function, as measured by the Unified Parkinson's Disease Rating Scale – Part III (UPDRS III) in people with Parkinson's experiencing OFF periods. Multiple secondary endpoints, including achievement of an ON state with maintenance through 60 minutes and Patient Global Impression of Change (PGIC), were supportive of the primary endpoint result. These findings are being presented at the International Congress of Parkinson's Disease and Movement Disorders (MDS), being held in Vancouver, British Columbia from June 4-8, 2017.

Acorda is developing CVT-301 as a treatment for symptoms of OFF periods in people with Parkinson's taking a carbidopa / levodopa regimen. OFF periods refer to the re-emergence of Parkinson's symptoms.

Key Efficacy and Safety Findings: Phase 3 SPAN-PD Study

The poster “Inhaled levodopa (CVT-301, 84-mg dose) significantly improves motor function during OFF periods in Parkinson's disease subjects: A Phase 3 Study (SPAN-PD)” (Poster #LBA34) highlighted findings from a 12-week,
placebo-controlled trial that enrolled 339 participants.

The study met its primary endpoint, with CVT-301 84 mg showing statistically significant improvement in motor function compared to placebo as measured by mean change in the UPDRS III at 30-minutes post-dose at Week 12 (-9.83 vs. -5.91; p = 0.009).

Secondary efficacy analyses were performed using a pre-specified hierarchy. The order of hierarchy was set based on probability of success, guided by the Phase 2b results. The primary endpoint (CVT-301 84 mg vs. placebo) was tested first for statistical significance. Upon achieving significance, the secondary endpoints were tested for CVT-301 84 mg vs. placebo followed by CVT-301 60 mg in the hierarchical order, as long as each preceding endpoint reached a significance level of P < 0.05. The hierarchical sequence did not reach statistical significance at Step 3. Unadjusted (nominal) p-values are presented below for all key secondary endpoints.

<table>
<thead>
<tr>
<th>Endpoint</th>
<th>Hierarchy Order</th>
<th>Δ</th>
<th>P-Value</th>
</tr>
</thead>
<tbody>
<tr>
<td>84 mg vs. placebo</td>
<td>Change in UPDRS III at 30 min (primary)</td>
<td>1</td>
<td>-3.92</td>
</tr>
<tr>
<td></td>
<td>% OFF to ON and remaining ON at 60 min</td>
<td>2</td>
<td>21.60</td>
</tr>
<tr>
<td></td>
<td>Change in UPDRS III at 20 min</td>
<td>3</td>
<td>-2.55</td>
</tr>
<tr>
<td></td>
<td>% subjects improved on PGIC</td>
<td>4</td>
<td>25.00</td>
</tr>
<tr>
<td></td>
<td>Change in UPDRS III at 10 min</td>
<td>5</td>
<td>-2.26</td>
</tr>
<tr>
<td></td>
<td>Change in PD diary OFF time</td>
<td>6</td>
<td>0.67</td>
</tr>
<tr>
<td>60 mg vs. placebo</td>
<td>Change in UPDRS III at 30 min</td>
<td>7</td>
<td>-3.07</td>
</tr>
<tr>
<td></td>
<td>% OFF to ON and remaining ON at 60 min</td>
<td>8</td>
<td>19.50</td>
</tr>
<tr>
<td></td>
<td>Change in PD diary OFF time</td>
<td>9</td>
<td>1.98</td>
</tr>
<tr>
<td></td>
<td>% subjects improved on PGIC</td>
<td>10</td>
<td>15.20</td>
</tr>
<tr>
<td></td>
<td>Change in UPDRS III at 10 min</td>
<td>11</td>
<td>0.97</td>
</tr>
<tr>
<td></td>
<td>Change in PD diary OFF time</td>
<td>12</td>
<td>-0.10</td>
</tr>
</tbody>
</table>

** Statistically significant on a nominal and adjusted basis
* Statistically significant on a nominal basis only

The most commonly reported adverse events in the CVT-301 84 mg group compared to the placebo group were: cough (14.9% vs. 1.8%, reported mostly once/subject), upper respiratory tract infection (6.1% vs. 2.7%), nausea (5.3% vs. 2.7%), sputum discoloration (5.3% vs. 0%) and dyskinesia (3.5% vs. 0.0%). When cough was reported, it was typically characterized as mild. Two of 114 participants receiving CVT-301 84 mg discontinued the study due to cough.

Key Safety and Exploratory Efficacy Findings: Long-Term Safety Study

In addition to the data presented at the MDS congress, Acorda announced interim data from an ongoing long-term safety study (CVT-301-005). This is a 52-week open-label study comparing treatment with CVT-301 84 mg with an observational control group. Participants were randomized in a 2:1 ratio into either the CVT-301 84 mg arm (n=271) or the observational control arm (n=127) of the study.

The primary objective of this study is to assess pulmonary function. Measures include Forced Expiratory Volume in 1 second (FEV1) and diffusing capacity of the lung for carbon monoxide (DLco).
There were no statistical differences in the mean changes in FEV1 and DLco from baseline to Week 52 between the CVT-301 84 mg and observational control groups.

The most common adverse events that were reported in any study arm at >5% were:

<table>
<thead>
<tr>
<th>Adverse Event</th>
<th>Observational Control (n=127)</th>
<th>CVT-301 84 mg (n=271)</th>
</tr>
</thead>
<tbody>
<tr>
<td>Cough</td>
<td>1 (0.8%)</td>
<td>35 (12.9%)</td>
</tr>
<tr>
<td>Nasopharyngitis</td>
<td>6 (4.7%)</td>
<td>17 (6.3%)</td>
</tr>
<tr>
<td>Dyskinesia</td>
<td>4 (3.1%)</td>
<td>15 (5.5%)</td>
</tr>
<tr>
<td>Fall</td>
<td>3 (2.4%)</td>
<td>14 (5.2%)</td>
</tr>
<tr>
<td>Bone Fracture (various types)</td>
<td>3 (2.4%)</td>
<td>14 (5.2%)</td>
</tr>
</tbody>
</table>

All reported fractures were judged by the investigators to be unlikely related or not related to study drug. Most reports of cough were mild (91%), none was severe. Three of 271 participants (1.1%) receiving CVT-301 discontinued the study due to cough.

Participants reporting serious adverse events (SAEs) were as follows: 13 (10.2%) in the observational control arm and 40 (14.8%) in the CVT-301 84 mg arm. Urinary tract infection occurred in four participants (1.5%) receiving CVT-301 84 mg. No other SAEs in the CVT-301 treatment group were reported at greater than 1%. There was one death in the study, a drowning in the CVT-301 84 mg group, judged by the investigator to be not related to study drug.

The study also included several uncontrolled, exploratory efficacy measurements that were only conducted in the CVT-301 arm of the study. Exploratory endpoints are hypothesis-generating. Findings included:

- At Week 52, the least-square mean of UPDRS III change from pre-dose at 30 minutes post-dose was -15.13 (n=130).
- 85% of participants (n=129) maintained an ON state 60 minutes post-dose at Week 52.
- 73% of participants (n=165) reported improvement as measured by PGIC at Week 52.
- The least-square mean reduction in daily OFF time among participants who completed the Week 52 visit (n=108) was 1.15 hours.
- Over the course of the study, the average daily usage of CVT-301 was 2.3 doses per day.

Acorda plans to file an NDA for CVT-301 with the U.S. Food and Drug Administration (FDA) by the end of the second quarter of 2017, and a Marketing Authorization Application (MAA) with the European Medicines Agency (EMA) by the end of the year.

**Investor Webcast Information**
The Company will host a webcast for investors to provide an overview of the CVT-301 clinical program on June 5, 2017 at 4:30 pm Eastern / 1:30 pm Pacific. This will include data being presented at the MDS Congress, as well as additional interim findings from a long-term safety study.

The webcast will include presentations by Matthew Stern, M.D., University of Pennsylvania, Peter LeWitt, M.D., Wayne State University School of Medicine, and Donald Grosset, M.D., Institute of Neurological Sciences, Queen Elizabeth University Hospital (Glasgow).

The webcast will be available on the Investor Events page of www.acorda.com and will be archived in the same location for replay. To participate via conference call, please dial 800-806-5484 (U.S.) or 416-340-2217 (international) and reference the access code 8170198#. An audio replay will be available until June 12, 2017 at 1:00 pm Eastern at 800-408-3053 (U.S.) or 905-694-9451 (international) using access code 5949682#.

About Parkinson's disease and OFF Periods

Approximately one million people in the U.S. and 1.2 million Europeans are diagnosed with Parkinson's disease (PD); OFF periods are experienced by approximately 350,000 in the U.S. and 420,000 in Europe.

Parkinson's is a progressive neurodegenerative disorder resulting from the gradual loss of certain neurons responsible for producing dopamine. It causes a range of symptoms including impaired movement, muscle stiffness and tremors. As PD progresses, people with Parkinson's experience OFF periods, which are characterized by the re-emergence of PD symptoms. This re-emergence can occur even when an individual's treatment regimen has been optimized.

OFF periods can be very disruptive to the lives of people with Parkinson's, their families and caregivers. OFF periods can increase in frequency and severity during the course of the disease.

About CVT-301 (levodopa inhalation powder) and ARCUS®

CVT-301 is a self-administered, inhaled levodopa (L-dopa) therapy in development for the treatment of symptoms of OFF periods in people with Parkinson's disease taking a carbidopa / levodopa regimen.

CVT-301 utilizes Acorda's investigational ARCUS® platform for inhaled therapeutics. CVT-301 was designed to deliver a precise dose of a dry powder formulation of L-dopa to the lung. Oral medication can be associated with variable onset of action, as the medicine is absorbed through the gastrointestinal (digestive) tract before reaching the brain. Inhaled treatments enter the body through the lungs and reach the brain, bypassing the digestive system.

About Acorda Therapeutics
Founded in 1995, Acorda Therapeutics is a biopharmaceutical 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 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 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., which will likely be materially adversely affected by the recently announced court decision in our litigation against filers of Abbreviated New Drug Applications (each, an “ANDA”) to market generic versions of 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 INBRIJA (CVT-301, levodopa inhalation powder), or any other acquired or in-licensed programs; we may not be able to complete development of, obtain regulatory approval for, or successfully market INBRIJA, any other products under development, or the products that we acquired with 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 press 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 press release.

Source: Acorda Therapeutics, Inc.

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