Theravance Biopharma, Inc. Announces Orphan Drug Designation Granted to Ampreloxetine for the Treatment of Symptomatic Neurogenic Orthostatic Hypotension in Patients with Multiple System Atrophy

May 9, 2023

DUBLIN, May 9, 2023 /PRNewswire/ -- Theravance Biopharma, Inc. ("Theravance Biopharma" or the "Company") (NASDAQ: TBPH) today announced that the US Food and Drug Administration (FDA) has granted Orphan Drug Designation (ODD) status to ampreloxetine for the treatment of symptomatic neurogenic orthostatic hypotension (nOH) in patients with multiple system atrophy (MSA). MSA is a rare neurodegenerative disorder in which patients suffer autonomic dysfunction affecting movement, balance, heart rate and blood pressure. Many patients with MSA suffer from the debilitating symptoms of nOH including dizziness, disrupted vision, muscle weakness, head and neck discomfort, and fatigue, which adversely affect quality of life.

"*We are very pleased to have received Orphan Drug status for ampreloxetine in the treatment of nOH in patients with MSA,*" said Rick E Winningham, Chief Executive Officer. "*Ampreloxetine has the potential to improve MSA patients' and their caregivers' quality of life significantly. We are diligently progressing our registrational Phase 3 study (CYPRESS) study of ampreloxetine for the treatment of symptomatic nOH in patients with MSA and today's Orphan Drug Designation status approval represents an important milestone towards our goal of bringing this potential therapy to patients with limited treatment options.*"

Ampreloxetine is a once-daily norepinephrine reuptake inhibitor discovered at Theravance Biopharma. Previous clinical studies have demonstrated that ampreloxetine increased norepinephrine levels, prevented a drop in blood pressure, and prevented symptom worsening in patients with MSA. Approximately 80% of the 50,000 MSA patients in the U.S. suffer from nOH.[1,2]

The Orphan Drug Act of 1983 (ODA) was created to incentivize the pharmaceutical and medical communities to develop products to treat rare diseases, defined as those for which the U.S. prevalence is less than 200,000 individuals. Under the ODA, companies can request Orphan Drug Designation status for an experimental therapy and stand to receive a 50% tax credit toward the cost of qualified clinical trial and seven years of marketing exclusivity, if approved. Orphan Drugs are also exempt from user fees associated with regulatory review.

About Ampreloxetine

Ampreloxetine, an investigational, once-daily norepinephrine reuptake inhibitor in development for the treatment of symptomatic neurogenic orthostatic hypotension (nOH) in patients with multiple system atrophy (MSA). The unique benefits of ampreloxetine treatment reported in MSA patients from Study 0170 included an increase in norepinephrine levels, a favorable impact on blood pressure, clinically meaningful and durable symptom improvements, and no signal for supine hypertension. The Company presented three scientific platform presentations at the 2022 American Autonomic Society meeting.[3]

About Multiple System Atrophy (MSA) and Symptomatic Neurogenic Orthostatic Hypotension (nOH)

MSA is a progressive brain disorder that affects movement and balance and disrupts the function of the autonomic nervous system. The autonomic nervous system controls body functions that are mostly involuntary. One of the most frequent autonomic symptoms associated with MSA is a sudden drop in blood pressure upon standing (nOH).[4] There are approximately 50,000 MSA patients in the US[1] and 70-90% of MSA patients experience nOH symptoms.[2] Despite available therapies, many MSA patients remain symptomatic with nOH.

Neurogenic orthostatic hypotension (nOH) is a rare disorder defined as a fall in systolic blood pressure of ⩾20 mm Hg or diastolic blood pressure of ⩾10 mm Hg, within 3 minutes of standing. Severely affected patients are unable to stand for more than a few seconds because of their decrease in blood pressure, leading to cerebral hypoperfusion and syncope. A debilitating condition, nOH results in a range of symptoms including dizziness, lightheadedness, fainting, fatigue, blurry vision, weakness, trouble concentrating, and head and neck pain.

About Theravance Biopharma

Theravance Biopharma, Inc.'s focus is to deliver *Medicines that Make a Difference*® in people's lives. In pursuit of its purpose, Theravance Biopharma leverages decades of expertise, which has led to the development of FDA-approved YUPELRI® (revefenacin) inhalation solution indicated for the maintenance treatment of patients with chronic obstructive pulmonary disease (COPD). Ampreloxetine, its late-stage investigational norepinephrine reuptake inhibitor in development for symptomatic neurogenic orthostatic hypotension, has the potential to be a first in class therapy effective in treating a constellation of cardinal symptoms in multiple system atrophy patients. The Company is committed to creating/driving shareholder value.

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

Forward-Looking Statements

This press release will contain certain "forward-looking" statements as that term is defined in the Private Securities Litigation Reform Act of 1995 regarding, among other things, statements relating to goals, plans, objectives, expectations and future events. Theravance Biopharma intends such forward-looking statements to be covered by the safe harbor provisions for forward-looking statements contained in Section 21E of the Securities Exchange Act of 1934, as amended, and the Private Securities Litigation Reform Act of 1995. Examples of such statements include statements relating to: the Company's goals, designs, strategies, plans and objectives, the Company's regulatory strategies and timing of clinical studies (including the data therefrom), the potential characteristics, benefits and mechanisms of action of the Company's product and product candidates and the Company's expectations for product candidates through development and potential regulatory approval and commercialization (including their differentiation from other products or potential products). These statements are based on the current estimates and assumptions of the management of Theravance Biopharma as of the date of this press release and are subject to risks, uncertainties, changes in circumstances, assumptions and other factors that may cause the actual results of Theravance Biopharma to be materially different from those reflected in the forward-looking statements. Important factors that could cause actual results to differ materially from those indicated by such forward-looking statements include, among others, risks related to: delays or difficulties in commencing, enrolling or completing clinical studies, the potential that results from clinical or non-clinical studies indicate the Company's product candidates or product are unsafe, ineffective or not differentiated, risks of decisions from regulatory authorities that are unfavorable to the Company, dependence on third parties to conduct clinical studies, delays or failure to achieve and maintain regulatory approvals for product candidates, risks of collaborating with or relying on third parties to discover, develop, manufacture and commercialize products, and risks associated with establishing and maintaining sales, marketing and distribution capabilities with appropriate technical expertise and supporting infrastructure, ability to retain key personnel, the impact of the Company's recent restructuring actions on its employees, partners and others, the ability of the Company to protect and to enforce its intellectual property rights, volatility and fluctuations in the trading price and volume of the Company's shares, and general economic and market conditions. Other risks affecting Theravance Biopharma are in the Company's Form 10-K filed with the SEC on March 1, 2023, and other periodic reports filed with the SEC. In addition to the risks described above and in Theravance Biopharma's filings with the SEC, other unknown or unpredictable factors also could affect Theravance Biopharma's results. No forward-looking statements can be guaranteed, and actual results may differ materially from such statements. Given these uncertainties, you should not place undue reliance on these forward-looking statements. Theravance Biopharma assumes no obligation to update its forward-looking statements on account of new information, future events or otherwise, except as required by law.

Contact:
investor.relations@theravance.com
650-808-4045

[1] UCSD Neurological Institute (25K-75K, with ~10K new cases per year); NIH National Institute of Neurological Disorders and Stroke (15K-50K).

[2] Delveinsight MSA Market Forecast (2023); Symptoms associated with orthostatic hypotension in pure autonomic failure and multiple systems atrophy, CJ Mathias (1999).

[3] November 2022, Biaggioni I, et al. Abstract 34 / Virtual Poster 106; Kaufmann H, et al. Abstract 33 / Virtual Poster 117; Freeman R, et al. Abstract 30 / Virtual Poster 4

[4] https://medlineplus.gov/genetics/condition/multiple-system-atrophy/

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