FDA Extends PDUFA Date of Biohaven's Troriluzole NDA for Rare Disease Spinocerebellar Ataxia

May 14, 2025

- Spinocerebellar Ataxia (SCA) is a rare, genetic, life-threatening neurodegenerative disease with no available treatment.

- Troriluzole has been granted Fast-Track, Orphan Drug Designation (ODD) and Priority Review from the FDA.

- Troriluzole would be the first and only FDA-approved treatment for SCA, if approved.

NEW HAVEN, Conn., May 14, 2025 /PRNewswire/ -- Biohaven Ltd. (NYSE: BHVN) (Biohaven or the Company), today announced that the Division of Neurology 1 within FDA's Office of Neuroscience informed the Company that they are extending the PDUFA date for the troriluzole new drug application (NDA) for the treatment of spinocerebellar ataxia (SCA) by three months to provide time for a full review of Biohaven's recent submissions related to information requests from the FDA. The Division also informed Biohaven that it is currently planning to hold an advisory committee meeting to discuss the application, but no date has been scheduled. The FDA did not raise any new concerns in the letter. The FDA's decision regarding the NDA is now expected in 4Q 2025.

Biohaven previously received Fast-Track, Orphan Drug Designation (ODD) and Priority Review from the FDA regarding troriluzole for SCA. Priority Review designation is assigned to applications for drugs that would offer a significant improvement over other available treatments for a given disorder or would provide a treatment option where none exists. In the case of SCA, a rare, genetic, neurodegenerative disease, troriluzole would be the first and only FDA-approved treatment for this life-threatening disorder.

Vlad Coric, M.D., Chairman and Chief Executive Officer of Biohaven stated, "SCA is a devastating neurodegenerative disease that has affected generations of families and has no current approved therapy. We are committed to bringing the first treatment to patients and families affected by SCA. The clinical data presented in the NDA show a highly favorable benefit-risk profile with troriluzole, a once-daily oral pill, slowing disease progression by 50-70%, as measured by the f-SARA scale, and reducing the risk of falls. We look forward to a meeting with the advisory committee to discuss troriluzole's potential to improve the lives of individuals with SCA. We are especially grateful to the patients, families, and leading neurologists who participated in our studies over an 8-year period and the broader SCA community and patient advocacy groups, including the National Ataxia Foundation, who supported the review of troriluzole as the first potential therapy for SCA."

The Company recently announced it has completed the FDA mid-cycle review meeting and regulatory inspections of Biohaven and key clinical research sites for troriluzole in the treatment of SCA. The mid cycle review concluded that there were no previously unidentified major safety concerns, and it does not appear a Risk Evaluation and Mitigation Strategy (REMS) is needed.

About Spinocerebellar Ataxia (SCA)
Spinocerebellar ataxia is a group of dominantly inherited neurodegenerative disorders characterized by progressive loss of voluntary motor control and atrophy of the cerebellum and brainstem. SCA affects approximately 15,000 people in the United States and 24,000 in Europe and the United Kingdom. Patients experience significant morbidity, including impaired gait leading to falls, loss of ambulation and progression to a wheelchair, inability to communicate due to speech impairment, difficulty swallowing, and premature death. While signs and symptoms can appear anytime from childhood to late adulthood, SCA typically presents in early adulthood and progresses over a number of years. Currently, there are no FDA-approved treatments and no cure for SCA.

About Troriluzole
Troriluzole is a new chemical entity (NCE) and third-generation novel prodrug that modulates glutamate, the most abundant excitatory neurotransmitter in the human body. The primary mode of action of troriluzole is reducing synaptic levels of glutamate. Troriluzole increases glutamate uptake from the synapse, by augmenting the expression and function of excitatory amino acid transporters located on glial cells that play a key role in clearing glutamate from the synapse. The glutamate modulating activity of troriluzole addresses the widely documented glutamate deregulation that underlies neurodegeneration and Purkinje cell dysfunction in patients with SCA. Troriluzole also has the potential to be developed in a number of other diseases associated with excessive glutamate. More information about troriluzole can be found at the Biohaven's website: **https://www.biohaven.com/pipeline/clinical-programs/glutamate/**.

About Biohaven
Biohaven is a biopharmaceutical company focused on the discovery, development, and commercialization of life-changing treatments in key therapeutic areas, including immunology, neuroscience, and oncology. The company is advancing its innovative portfolio of therapeutics, leveraging its proven drug development experience and multiple proprietary drug development platforms. Biohaven's extensive clinical and preclinical programs include Kv7 ion channel modulation for epilepsy and mood disorders; extracellular protein degradation for immunological diseases; TRPM3 antagonism for migraine and neuropathic pain; TYK2/JAK1 inhibition for neuroinflammatory disorders; glutamate modulation for OCD and SCA; myostatin inhibition for neuromuscular and metabolic diseases, including SMA and obesity; antibody recruiting bispecific molecules and antibody drug

conjugates for cancer. For more information, visit **www.biohaven.com.**

Forward-looking Statements
This news release includes forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995. The use of certain words, including "continue", "plan", "will", "believe", "may", "expect", "anticipate" and similar expressions, is intended to identify forward-looking statements. Investors are cautioned that any forward-looking statements, including statements regarding the future development, timing and potential marketing approval and commercialization of development candidates, including the potential FDA approval and commercialization of troriluzole for SCA, are not guarantees of future performance or results and involve substantial risks and uncertainties. Actual results, developments and events may differ materially from those in the forward-looking statements as a result of various factors including: the expected timing, commencement and outcomes of Biohaven's planned and ongoing clinical trials; the timing of planned interactions and filings with the FDA; including those regarding the potential FDA approval of Troriluzole for SCA; the timing and outcome of expected regulatory filings; complying with applicable U.S. regulatory requirements; the potential commercialization of Biohaven's product candidates; the potential for Biohaven's product candidates to be first approved therapies; and the effectiveness and safety of Biohaven's product candidates. Additional important factors to be considered in connection with forward-looking statements are described in Biohaven's filings with the Securities and Exchange Commission, including within the sections titled "Risk Factors" and "Management's Discussion and Analysis of Financial Condition and Results of Operations". The forward-looking statements are made as of the date of this news release, and Biohaven does not undertake any obligation to update any forward-looking statements, whether as a result of new information, future events or otherwise, except as required by law.

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