



## Biohaven's Taldefgrobep Alfa Receives EU Orphan Drug Designation for Spinal Muscular Atrophy

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- Taldefgrobep alfa, in Phase 3 global clinical development for Spinal Muscular Atrophy, granted EU Orphan Drug Designation in addition to previously receiving Fast Track and Orphan Drug Designation in the US

NEW HAVEN, Conn., July 31, 2023 /PRNewswire/ -- Biohaven Ltd. (NYSE: BHVN; "Biohaven") announced today that it received orphan medicinal product designation from the European Commission (EC) for taldefgrobep alfa, a novel anti-myostatin adnectin, for the treatment of spinal muscular atrophy (SMA). Orphan Designation highlights the potential for taldefgrobep to deliver significant benefit for people with SMA and provides regulatory and commercial incentives including a reduction in regulatory fees associated with protocol reviews and scientific advice, along with an additional ten-years of market protection. Biohaven previously received fast track and orphan drug designation from the FDA for taldefgrobep in the treatment of SMA.



SMA is a rare, progressively debilitating motor neuron disease in which development and growth of muscle mass are compromised, resulting in progressive weakness and muscle atrophy, reduced motor function, impaired quality of life and often death. Inhibition of myostatin, a naturally occurring protein that limits skeletal muscle growth, an important process in healthy muscular development, is a promising therapeutic strategy for SMA.

Taldefgrobep has the potential to be a novel therapy to enhance muscle function in SMA by blocking myostatin, when used in combination with currently available disease modifying therapies that help preserve motor neurons. Taldefgrobep's novelty in a field of myostatin inhibitors is based on its unique, dual mechanism of action. It binds to myostatin to lower overall myostatin levels and also functions as a receptor antagonist, thereby blocking myostatin signaling in skeletal muscles.

Irfan Qureshi, MD, Chief Medical Officer, Biohaven commented, "We are delighted that the European Commission granted orphan drug designation for taldefgrobep alfa for the treatment of SMA. Children and adults living with SMA experience significant muscle weakness and functional impairments affecting their quality of daily life, and a substantial unmet medical need persists. We are excited about the potential for taldefgrobep alfa to improve the lives of patients and families affected by SMA."

As a leader in innovative trials addressing neurodegenerative diseases, Biohaven is currently enrolling a Phase 3 clinical trial of taldefgrobep in SMA: A Study to Evaluate the Efficacy and Safety of Taldefgrobep Alfa in Participants with Spinal Muscular Atrophy (RESILIENT) (NCT05337553).

### About Taldefgrobep alfa

Taldefgrobep alfa (also known as BHV2000) is a modified adnectin designed to specifically bind to myostatin (GDF-8). Taldefgrobep is a fully human anti-myostatin recombinant protein that lowers free myostatin and acts as an Activin 2b receptor antagonist with the myostatin-taldefgrobep complex. Adnectins are an established proprietary protein therapeutic class based on human fibronectin, an extracellular protein that is naturally abundant in human serum.

### About Spinal Muscular Atrophy (SMA)

Spinal muscular atrophy (SMA) is a rare genetic neurodegenerative disorder characterized by the loss of motor neurons, atrophy of the voluntary muscles of the limbs and trunk and progressive muscle weakness that is often fatal and typically diagnosed in young children. The underlying pathology of SMA is caused by insufficient production of the SMN (survival of motor neuron) protein, essential for the survival of motor neurons, and is encoded by two genes, SMN1 and SMN2. In the U.S., SMA affects approximately 1 in 11,000 births, and about 1 in every 50 Americans is a genetic carrier.

### About Biohaven

Biohaven is a global clinical-stage biopharmaceutical company focused on the discovery, development and commercialization of life-changing therapies to treat a broad range of rare and common diseases. Biohaven's experienced management team brings with it a track record of delivering new drug approvals for products for diseases such as migraine, depression, bipolar disorder and schizophrenia. Biohaven is advancing a pipeline of therapies for diseases with little or no treatment options, leveraging its proven drug development capabilities and proprietary platforms, including Kv7 ion channel modulation for epilepsy and neuronal hyperexcitability, glutamate modulation for obsessive-compulsive disorder and spinocerebellar ataxia, myostatin inhibition for neuromuscular diseases, and brain-penetrant TYK2/JAK1 inhibition for immune-mediated brain disorders. Biohaven's portfolio of early- and late-stage product candidates also includes discovery research programs focused on TRPM3 channel activation for neuropathic pain, CD-38 antibody recruiting, bispecific molecules for multiple myeloma, antibody drug conjugates (ADCs), and targeted extracellular protein degrader platform technology (MoDEs™ platform) with potential application in neurological disorders, cancer, and autoimmune diseases. For more information, visit [www.biohaven.com](http://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 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 Food and Drug Administration; 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 in class 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 new 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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