Biohaven's Taldefgrobep Alfa Receives FDA Fast Track Designation for Spinal Muscular Atrophy

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- Taldefgrobep alfa, a myostatin-targeting biologic investigational agent, in Phase 3 development to increase muscle mass for Spinal Muscular Atrophy patients now granted Fast Track in addition to previously receiving Orphan Drug Designation in the US

NEW HAVEN, Conn., Feb. 21, 2023 /PRNewswire/ -- Biohaven Ltd. (NYSE: BHVN; "Biohaven") announced today that it received Fast Track designation from the U.S. Food and Drug Administration (FDA) for taldefgrobep alfa, a novel anti-myostatin adnectin, for the treatment of spinal muscular atrophy (SMA). Fast Track designation enables important new drugs to reach patients earlier by facilitating more frequent communications with the FDA and expeditious review of a drug which treats a serious condition and fills an unmet medical need. Biohaven previously received 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 potential therapeutic target for SMA.

Taldefgrobep has the potential to be a novel therapy to be used in combination with disease modifying therapies to enhance muscle function by blocking myostatin activity. Taldefgrobep's novelty in a field of myostatin inhibitors is based on its mechanism of action. It binds to myostatin to both lower overall myostatin levels and also function as a receptor antagonist, thereby blocking myostatin signaling in skeletal muscles.

Lindsey Lee Lair, M.D., M.B.A., F.A.A.N, Vice President, Clinical Development, Biohaven commented, "We are very pleased the FDA granted Fast Track 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 life daily, 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."

Karen Chen, Ph.D., CEO, SMA Foundation, added, "Fast Track designation from the FDA underscores the high unmet medical need in SMA and supports the need for additional novel combination therapies in children and adults living with this progressive neurologic disease. The SMA Foundation is heavily investing in supporting the development of additional muscle and neuromuscular regenerative medicine therapies that provide functional benefits to patients. We are happy to see the commitment of health authorities to rapidly advance the development of new therapies"

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 for people with debilitating neurological and neuropsychiatric diseases, including rare disorders. The company 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 and myostatin inhibition for neuromuscular diseases. 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 extracellular target degrader platform technology (MoDE™) with potential application in neurological disorders,

cancer, and autoimmune diseases.

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 "believe", "may" and "will" and similar expressions, are intended to identify forward-looking statements. These forward-looking statements involve substantial risks and uncertainties, including statements that are based on the current expectations and assumptions of Biohaven's management about taldefgrobep alfa as treatment for patients with neuromuscular disease. 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. 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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