Biohaven Completes Enrollment in Pivotal Phase 3 Study of Taldefgrobep Alfa in Spinal Muscular Atrophy

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- Taldefgrobep alfa is the only myostatin inhibitor in clinical development that targets both myostatin and activin A signaling, two key regulators of muscle mass and adipose tissue

*- **RESILIENT**, the pivotal clinical trial in spinal muscular atrophy, was designed to test the efficacy and safety of taldefgrobep alfa as adjunctive therapy to increase muscle in SMA patients treated with standard of care nusinersen, risdiplam or onasemnogene abeparvovec-xioi*

NEW HAVEN, Conn., Sept. 14, 2023 /PRNewswire/ -- Biohaven Ltd. (NYSE: BHVN) ("Biohaven"), 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, today announced completing enrollment in RESILIENT, the Phase 3 pivotal study of taldefgrobep alfa in spinal muscular atrophy (SMA).

Lindsey Lair, MD, Biohaven Vice President, Clinical Development Lead for the SMA program stated, "We are thrilled to complete enrollment in this pivotal trial for SMA as it brings us one step closer to advancing a novel muscle targeting therapy for patients with SMA. Despite recent advances in SMA genetic treatments, patients still experience weakness and impairments in quality of life that can be alleviated by enhancing muscle mass and function, on top of what is delivered by current standard of care treatments." Dr. Lair added, "Our team has been inspired by the entire global SMA community of clinicians, patients and family members – we are particularly grateful to the patients and investigators who helped us complete enrollment ahead of our timelines."

Taldefgrobep is an investigational, muscle-targeted recombinant protein with the potential to enhance muscle mass and strength in people living with SMA when used in combination with other approved treatments. Taldefgrobep targets myostatin, a natural protein that limits skeletal muscle growth. Myostatin inhibition is a potential therapeutic strategy for children and adults with a range of neuromuscular conditions for whom active myostatin can limit the skeletal muscle growth needed to achieve developmental and functional milestones.

RESILIENT is a Phase 3 placebo-controlled, double-blind trial designed to evaluate the efficacy and safety of taldefgrobep at 48 weeks as an adjunctive therapy for participants who are already taking a stable dose of nusinersen, risdiplam and/or have a history of treatment with onasemnogene abeparvovec-xioi, compared to placebo. The study is not restricted nor limited to patients based on ambulatory status or classification of SMA.

Taldefgrobep was granted EU Orphan Drug Designation, along with both Fast Track and Orphan Drug Designation by the US FDA.

"While we have had good progress with current therapies, a high unmet need for safe and effective supportive treatments for SMA remains, as many patients still experience significant weakness and reduced levels of functioning. We appreciate the partnership of investigators, patients, and researchers to expedite the development of new efficacious therapies that will work in combination with current options to help restore muscle strength and function," said Kenneth Hobby, President, Cure SMA.

Biohaven targeted randomizing approximately 180 patients in this global trial conducted in 9 countries. The primary objective is to determine the safety and efficacy of taldefgrobep alfa compared to placebo after 48 weeks of subcutaneous administration as determined by change from baseline in the 32 item Motor Function Measure (MFM-32) total score. Additional details about the trial can be found at SMATrial.com or https://clinicaltrials.gov/NCT05337553.

About Taldefgrobep alfa
Taldefgrobep alfa (BHV-2000) is a fully human recombinant protein specifically designed to inhibit the signaling of myostatin and activin A; two key regulators of muscle and adipose tissue. Taldefgrobep binds myostatin and acts as an Activin 2b receptor antagonist. Taldefgrobep's novel mechanism of action offers the potential for meaningful reductions in fat mass, increased lean mass, and improvements in multiple metabolic parameters.

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. Globally, SMA affects approximately 1 in 11,000 births, and about 1 in every 50 individuals 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. 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 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 and CD-38 antibody recruiting, bispecific molecules for multiple myeloma. More information about Biohaven is available at 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 "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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