Biohaven Provides Overview of Clinical Progress, Regulatory Updates, and Pipeline Developments at R&D Day

May 31, 2023

- *Submitted a new drug application (NDA) for troriluzole in Spinocerebellar Ataxia Type 3 (SCA3) to U.S. FDA in 2Q2023, marking the team's fourth NDA in approximately 3 years*
- *Released additional data from Kv7 platform, including Phase 1 safety data by dose groups for BHV-7000 that further validates differentiated profile*
- *Projected Phase 3 Spinal Muscular Atrophy trial to complete enrollment in 2023*
- *Initiated Phase 1 study of brain penetrant TYK2/JAK1 inhibitor, BHV-8000, and anticipate beginning Phase 2 trial in Parkinson's disease next year*
- *Highlighted robust pipeline with multiple INDs planned to be filed within the next year, including pan IgG degrader for multiple immune-mediated diseases in 2023*

NEW HAVEN, Conn., May 31, 2023 /PRNewswire/ -- Biohaven Ltd. (NYSE: BHVN) ("Biohaven"), a global clinical-stage biopharmaceutical company focused on the discovery, development and commercialization of life-changing therapies for people with debilitating diseases, including ultra-rare disorders, will provide an overview of clinical progress, regulatory updates, and pipeline developments at an in-person R&D Day today, concurrently with the Yale Ventures' Innovation Summit 2023 taking place on May 31-June 1. Members of Biohaven's senior management team and key opinion leaders will meet with investors and research analysts.

The clinical progress, regulatory updates, and pipeline developments to include:

- **Troriluzole in SCA:** Submitted New Drug Application (NDA) to the U.S. FDA for troriluzole for the treatment of spinocerebellar ataxia type 3 (SCA3), an ultra-rare, genetically-defined, neurodegenerative disease associated with progressive disability, frequent falls, loss of ambulation, speech and swallowing impairment, and premature death that is the most common SCA genotype worldwide.
    - NDA supported by consistent treatment benefits observed in patients with genotype SCA3 in Study BHV4157-206 across multiple outcome measures including the change from baseline f-SARA at Week 48, CGI-I total score at Week 48, and a robust reduction in fall risk over the study period. A rigorous analysis by genotype was possible as patients were randomized by genotype strata at baseline prior to randomization in the pivotal Phase 3 48-week, double-blind, placebo-controlled phase of Study BHV4157-206.
    - SCA3 represented 41% of study participants, consistent with being the most common subtype. SCA3 affects approximately 10,600 people in North America and in the EU and Japan.
    - NDA further supported by a composite scale development (SCACOMS) and analysis of Study BHV4157-206, and confirmatory evidence of efficacy provided by data from the 3-year, long-term open-label (OLE) extension phase of two studies (BHV4157-206 and BHV4157-201) using a Matching Adjusted Indirect Comparison (MAIC) to an external control group.
    - Biohaven has previously received Fast-Track and Orphan drug designation (ODD) from the FDA, and ODD from the European Medicines Agency, for troriluzole in SCA.

- **Kv7 Platform:** Highlighted progress and broad potential of Kv7 platform, including ongoing and planned development of BHV-7000 (Kv7.2/3 activator):
    - Phase 1 EEG study with BHV-7000 in 1H2023.
    - Pivotal studies with BHV-7000 in focal epilepsy and bipolar disorder planned to initiate in 2H2023.
    - Burgeoning evidence for therapeutic benefits of targeting Kv7 in diverse, high unmet need indications.

- **Bispecific Platform:** Provided updates regarding planned INDs for targeted extracellular protein degradation franchise (including IgG, IgA, and autoantibody-specific degrader programs) and next-generation antibody-drug conjugate (ADC)

technologies;
    - IND application for novel IgG degrader, BHV-1300, on track for submission in 2023.

- **TYK2/JAK1 Inhibition in Immune-Mediated Brain Disorders:** Began dosing with BHV-8000 (an oral, brain-penetrant, dual TYK2/JAK1 inhibitor) in a Phase 1 study in normal healthy volunteers.

- **Taldefgrobep Alfa:**
    - In Spinal Muscular Atrophy: Enrollment of approximately 225 patients in global Phase 3 trial now anticipated to complete in 2023.
    - In Metabolic Disorders: Planned Phase 2 trial initiation in 2H2023.

Vlad Coric M.D., CEO and Chairman of Biohaven, commented, "Today's R&D Day review of our robust pipeline highlights Biohaven's continued dedication to advance novel therapeutics for brain disorders and builds upon our team's successes with the prior approvals of Nurtec® ODT and Zavzpret™ that have changed the treatment paradigm in migraine."

"The NDA we submitted for troriluzole for SCA3 represents approximately seven years of effort by the Biohaven team to bring forward a potentially new treatment for this ultra-rare disease. Advancing a new investigational drug for a rare disease that has no current therapy is a multi-year process that is the culmination of not only our own internal efforts but also close coordination with patient advocacy groups including the National Ataxia Foundation (NAF), leading academic researchers and regulatory agencies. Rare brain diseases are particularly challenging to research given relatively small populations of patients to run / participate in clinical trials, the need to rely on real-world data and often a lack of standardized ratings scales or biomarkers. We could not have advanced the SCA program this far without the leadership from the National Ataxia Foundation that has supported basic science research as well as the development of natural history cohorts in SCA that serve as an external control for longitudinal studies. The Biohaven clinical trials in SCA were a first of its kind in this area and utilized a newly developed rating scale (the functional SARA or f-SARA) that was developed in close consultation with the FDA using standard regulatory pathways to elucidate this new scale. We look forward to interactions with the US FDA, EMA and other regulatory agencies across the globe as our submissions advance in the review process," Dr. Coric added.

"We are pleased that our decades-long investment in ataxia research and understanding of disease progression has accelerated treatment development for SCA," said Andrew Rosen, Executive Director of the National Ataxia Foundation. "Thank you to all NAF members who participated in these important trials."

Bruce Car PhD, Biohaven's CSO, stated, "Given the lack of efficacious therapies for many brain disorders, we must urgently evaluate new mechanisms and approaches to change the treatment paradigm in this therapeutic area. Our R&D Day today demonstrates the commitment that we have given to this effort with exciting new approaches ranging from our submission of a glutamate modulator in SCA to immune modulation using small molecule degraders to ion channels targeting agents for epilepsy and mood disorders, as well as a biologic treatment to enhance muscle growth in SMA. Our scientific and clinical team is poised to file multiple new INDs from our deep pipeline and complete late-stage clinical trials in the next couple of years. It is an exciting time at Biohaven for our passionate employees, patients and investors as we advance these novel investigational drugs in our pipeline."

The Research & Development Day presentations will be made available following the conclusion of the program on https://ir.biohaven.com/events-presentations/events.

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 diseases, including rare disorders. 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. 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, 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 (MoDE™) with potential application in neurological disorders, cancer, and autoimmune diseases. 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 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.

Investor Contact:

Jennifer Porcelli
Vice President, Investor Relations
jennifer.porcelli@biohavenpharma.com
+1 (201) 248-0741

Media Contact:
Mike Beyer
Sam Brown Inc.
mikebeyer@sambrown.com
+1 (312) 961-2502

View original content to download multimedia:https://www.prnewswire.com/news-releases/biohaven-provides-overview-of-clinical-progress-regulatory-updates-and-pipeline-developments-at-rd-day-301838058.html

SOURCE Biohaven Ltd.