TRIRILUZOLE did not differentiate from placebo on the primary endpoint or key secondary outcome measure at the end of the 8-week randomization phase.

Placebo response rates in this study were higher than expected compared to prior European randomized controlled trials in SCA.

Tririluzole was well tolerated and safely administered in this study population.

Biohaven continues to drive development of its broader portfolio including CGRP-antagonists for migraine and other glutamate modulating agents.

Biohaven affirms robust recruitment of its two Phase 3 CGRP antagonist efficacy trials and expects topline results in the first quarter of 2018.

Biohaven maintains a strong fiscal position, having reported $204.3 million in cash as of June 30, 2017, which is expected to support the Company’s clinical programs and operations for over 12 months from today.

New Haven, Connecticut (NYSE: BHVN) October 2, 2017 – Biohaven Pharmaceutical Holding Company Ltd. today reported topline results from its Phase 2/3 clinical trial evaluating tririluzole compared to placebo for the treatment of patients with spinocerebellar ataxia (SCA). The eight-week trial dosed 141 adult SCA patients at 18 centers in the United States. In this trial, tririluzole did not differentiate from placebo on the primary endpoint of the mean change from baseline on the Scale for the Assessment and Rating of Ataxia (SARA) after eight weeks of treatment.

After eight weeks of treatment, tririluzole treated subjects demonstrated an improvement of -0.81 points [95% CI: -1.4 to -0.2] on the SARA versus -1.05 points [95% CI: -1.6 to -0.4] improvement in placebo, p-value = 0.52. Placebo response in this genetically defined disorder was higher than expected based upon prior European randomized controlled trials in SCA (Romano et al 2015; Ristori et al 2010).

In addition, tririluzole did not differentiate on the key secondary efficacy endpoint of Patient’s Global Impression of Change (PGI-C). 50.8% of subjects treated with tririluzole considered themselves improved versus 59.1% treated with placebo, p-value = 0.28.

However, tririluzole did demonstrate a favorable safety and tolerability profile, with no drug-related serious adverse events and low discontinuation rates due to adverse events. Tririluzole has shown absence of a negative food effect, optimized oral bioavailability and no pattern of clinically significant effects on liver function, presenting a profile that appears distinct from what is described for riluzole in its U.S. prescribing information.

Although this study failed to demonstrate acute symptomatic effects of tririluzole in SCA, the long-term, open-label, extension phase is ongoing. This extension phase will allow for potential signal detection at later time points. Topline data from the extension phase is expected in 4Q2018.

“We are obviously disappointed that today’s topline clinical results do not support continued development of tririluzole as a symptomatic agent for patients with SCA, a devastating neurologic disorder for which novel treatments are urgently needed. This was the largest SCA clinical trial performed to date and important knowledge has been generated -- we plan to share our data with the
ataxia clinical leaders and the National Ataxia Foundation to help refine clinical trials in this therapeutic area. We thank the patients and clinical investigators who participated in this program and sincerely appreciate all that they have done to see this study through completion,” stated Vlad Coric, M.D., Chief Executive Officer of Biohaven.

Susan L. Perlman, M.D., Clinical Professor of Neurology and Director of the Ataxia Center and HD Center of Excellence at UCLA, stated, “Biohaven has accomplished something extremely important for patients with spinocerebellar ataxia (a chronic, progressive, incurable neurogenetic disorder) and for the doctors and scientists who work with these patients and families. The company mobilized 18 ataxia clinical research centers from around the United States, engaged them and trained them in a complex drug trial protocol, and was able to complete the protocol in a timely fashion and with excellent participation and safety outcomes. Biohaven is the first U.S. pharmaceutical company to advance into these uncharted waters for spinocerebellar ataxia and will lead the way for others in the future.”

Robert Berman, M.D., Chief Medical Officer of Biohaven added, “The Biohaven team had hoped this trial would bring a long-awaited treatment for patients with SCA and we are grateful for how the community of patients and researchers rallied around this trial, allowing us to enroll the study in under six months. While the efficacy results were unfortunately disappointing, this study did demonstrate important characteristics of trigriluzole, including a favorable safety and tolerability profile. For the first time, trigriluzole was dosed chronically for over 24 weeks in some patients.”

Dr. Coric continued, “We have a deep portfolio of clinic-ready compounds. With a strong cash position, we will continue to deploy our resources in what we believe is a cost effective and data driven fashion. Our team remains focused on progressing our other drug candidates within the CGRP (rimegepant and BHV-3500) and glutamate modulation platforms (BHV-0223 and BHV-5000).”

In its CGRP platform, the company anticipates a number of important upcoming milestones. Enrollment and randomization continue to proceed strongly in both of Biohaven’s Phase 3 clinical trials of rimegepant for the acute treatment of migraine, and the company expects to report topline results in the first quarter of 2018. The long-term safety study is expected to complete in the fourth quarter of 2018 to support a potential first half 2019 NDA submission. Biohaven also expects to file an IND with the FDA later this year for BHV-3500 for the treatment of migraine.

In its glutamate-modulation platform, Biohaven anticipates commencing a bioequivalence study for BHV-0223 for the treatment of patients with amyotrophic lateral sclerosis (ALS) in the fourth quarter of 2017. With regard to BHV-5000, a low trapping NMDA receptor antagonist in-licensed from AstraZeneca, the company continues to optimize its formulation to support a Phase 1 pharmacokinetic trial in Rett syndrome, as well as other neuropsychiatric indications. Biohaven will also continue its program to evaluate trigriluzole in non-movement disorders where the pathophysiology is distinct from SCA. On September 28, 2017, the FDA notified Biohaven that it may proceed with its Phase 2/3 trial of trigriluzole in OCD; the study is scheduled to enroll a first patient in the coming weeks.

About Biohaven

Biohaven is a clinical-stage biopharmaceutical company with a portfolio of innovative, late-stage product candidates targeting neurological diseases, including rare disorders. Biohaven has combined internal development and research with intellectual property licensed from companies and institutions including Bristol-Myers Squibb Company, AstraZeneca AB, Yale University, Catalent, Rutgers, ALS
Biopharma LLC and Massachusetts General Hospital. Currently, Biohaven’s lead development programs include multiple compounds across its CGRP receptor antagonist and glutamate modulation platforms. Biohaven’s common shares are listed on the New York Stock Exchange and traded under the ticker symbol BHVN. More information about Biohaven is available at www.biohavenpharma.com.

Forward-Looking Statements

This news release includes forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995. These forward-looking statements involve substantial risks and uncertainties, including statements that are based on the current expectations and assumptions of the Company’s management. All statements, other than statements of historical facts, included in this press release, including statements regarding the Company’s expectations for the timing and reporting of data from its ongoing and planned clinical trials, the timing of regulatory filings and development timelines and the potential benefits of trigriluzole for patients with non-movement disorder indications are forward-looking statements. The use of certain words, including the words “believe,” “continue,” “expect”, “may” and “will” and similar expressions are intended to identify forward-looking statements. The Company may not actually achieve the plans and objectives disclosed in the forward-looking statements and you should not place undue reliance on the Company’s forward-looking statements. Various important factors could cause actual results or events to differ materially from those that may be expressed or implied by our forward-looking statements, including the significant risks and uncertainties regarding the future clinical success and regulatory pathway for the company’s product candidates. Additional important factors to be considered in connection with forward-looking statements are described in the “Risk Factors” section of the Company’s Quarterly Report on Form 10-Q filed with the Securities and Exchange Commission on August 14, 2017. The forward-looking statements are made as of this date and the Company 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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