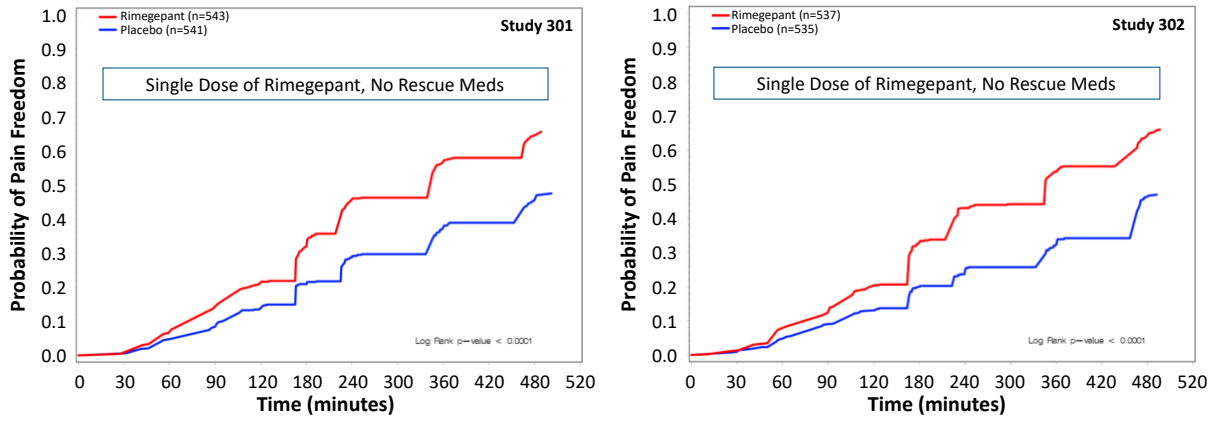
Biohaven Announces Successful Achievement of Both Co-Primary Regulatory Endpoints in Two Pivotal Phase 3 Trials of Rimegepant an Oral CGRP Receptor Antagonist for the Acute Treatment of Migraine

- Rimegepant met both registrational co-primary endpoints (pain freedom and freedom from most bothersome symptom at 2 hours) in two pivotal Phase 3 trials
- Results are statistically significant and clinically meaningful across multiple outcome measures
- A single dose of rimegepant, without any rescue medications, was superior to placebo for pain freedom and pain relief at 2 hours post-dosing with a profile of increasing improvement beyond 2 hours
- Rimegepant was well tolerated and demonstrated a liver safety profile similar to placebo (1 placebo and 1 rimegepant treated patient with liver function tests (LFTs) > 3x ULN, no rimegepant treated patients with > 5x, > 10x or > 20x ULN)
- Biohaven is on schedule to submit an NDA for rimegepant in 2019
- Company to host conference call and webcast with slides today at 8:30 am ET

NEW HAVEN, Connecticut, March 26, 2018 -- Biohaven Pharmaceutical Holding Company Ltd. (NYSE: BHVN) today announced positive top-line results from both of its two Phase 3 clinical trials (BHV3000-301 and BHV3000-302) of rimegepant (formerly known as BHV-3000), an oral CGRP receptor antagonist for the acute treatment of migraine. In each trial, rimegepant met the co-primary efficacy endpoints of superiority to placebo, at two hours post-dose, on pain freedom and freedom from the most bothersome symptom (MBS) (**Figure 1, Table 1**). Even without additional rimegepant dosing, or use of rescue medications, these studies showed an early separation from placebo and a profile of continued improvement over the dosing interval. Overall, efficacy and safety results were consistent across both Phase 3 trials.

Figure 1: Co-Primary Endpoint of Pain Freedom Met in Both Studies



The percent of patients experiencing pain freedom between 0 to 8 hours after dosing is shown in the Kaplan-Meier curves in Figure 1. Pain freedom at 2 hours for rimegepant treated subjects in Study 301 and 302 was 19.2% and 19.6%, respectively, versus 14.2% ($p < 0.03$) and 12.0% ($p < 0.001$) in the placebo treated groups. The magnitude of the treatment effect over placebo at 2 hours or later, as shown in the curves, ranged from 5% to 19% in Study 301 and 7% to 22% in Study 302. This continued improvement in pain freedom was observed in subjects who took a single dose of rimegepant without use of rescue medications.

Patients treated with rimegepant also achieved statistically significant benefits, as compared to placebo at 2 hours post-dose, in freedom from the MBS (selected from photophobia, phonophobia or nausea). Freedom from the MBS for rimegepant treated subjects in Study 301 and 302 were 36.6% and 37.6%, respectively, versus 27.7% ($p < 0.002$) and 25.2% ($p < 0.0001$) for placebo (**Table 1**).

Table 1: Co-Primary Endpoint of Freedom from Most Bothersome Symptom (MBS) Met in Both Studies

Study	2 Hour Endpoint	Rimegepant (N=543)	Placebo (N=541)	p-value
301	Freedom from MBS[1]	36.6%	27.7%	< 0.002
	2 Hour Endpoint	**Rimegepant** (N=537)	**Placebo** (N=535)	**p-value**
302	Freedom from MBS[1]	37.6%	25.2%	< 0.0001

[1]Most Bothersome Symptom of Photophobia, Phonophobia or Nausea.

In addition to efficacy observed on the co-primary registrational endpoints, onset of pain relief (defined as transitioning from moderate-to-severe pain to either no-pain or mild-pain) was observed early after rimegepant treatment with numerical separation evident between 30-45 minutes post-dosing. By two hours in both studies, after a single dose, pain relief was achieved in over 55% of rimegepant treated subjects. Pain relief is a clinically important secondary endpoint, often associated with reduced disability due to migraine attacks.

Subjects with difficult-to-treat migraines, as defined by the need for prophylactic treatments including Botox®, achieved improvements similar to the overall population in pain freedom, MBS, and pain relief at 2 hours.

Rimegepant was found to be both safe and well-tolerated in the two Phase 3 studies with a safety profile similar to placebo. In particular, pooled liver function test (LFT) results showed that rimegepant was no more likely than placebo to increase aminotransferase (ALT or AST) levels above the upper limit of normal (ULN) (**Table 2**). One patient treated with placebo and one patient treated with rimegepant showed LFTs > 3x ULN. No patients in either trial showed LFT elevations > 5x, > 10x or > 20x ULN. No patients in either trial experienced elevations in bilirubin > 2x ULN. No single adverse event (AE) occurred with an incidence higher than 2% and overall AE rates in the rimegepant group were similar to placebo. The most common AEs (pooled analysis across both studies) seen in patients treated with rimegepant were similar to placebo (nausea 1.4% and 1.1%, respectively; UTI 1% and 0.7%, respectively).

Table 2: Pooled Liver Function Test (LFT) Profile: Rimegepant was Similar to Placebo in Both Studies

Complete Dataset of LFT Results from Study 301 and Study 302*

ALT or AST	Placebo (n=1092)	Rimegepant (n=1089)
> ULN[1]	31 (2.8%)	23 (2.1%)
> 3x ULN	1 (0.1%)	1 (0.1%)
> 5x ULN	0.0%	0.0%
> 10x ULN	0.0%	0.0%
> 20x ULN	0.0%	0.0%

[1]Upper limit of normal; ALT: alanine aminotransferase; AST: aspartate aminotransferase

*No bilirubin elevations > 2x ULN across both Studies 301 and 302

Vlad Coric, M.D., Chief Executive Officer of Biohaven, commented, “The topline data from our two pivotal trials show that a single, oral dose of rimegepant has the potential to be an effective and safe acute treatment for migraine, addressing both pain and most bothersome symptoms without the need for repeat dosing or rescue medicines. By combining positive efficacy results and a favorable safety profile with ease of oral dosing, we believe that rimegepant will represent a significant improvement over existing treatment options. We thank the patients, investigators and dedicated Biohaven employees who have worked so hard to advance this drug candidate to this important milestone.”

Richard B. Lipton, M.D., Vice Chair of Neurology, Professor of Epidemiology and Population Health and Director of the Montefiore Headache Center, at the Albert Einstein College of Medicine, and Chair of

Biohaven's CGRP Scientific Advisory Board added, "Clinicians have long awaited novel treatment modalities for migraine. The results from these two studies are very exciting as rimegepant met its regulatory endpoints and also showed important clinical benefit to patients."

Declan Doogan, M.D., Chairman of Biohaven's board of directors, commented, "The results of these studies show consistent efficacy, together with an excellent safety profile, and demonstrate that rimegepant has the potential to be a major new therapeutic addition as an oral agent for people with migraine. I have great confidence this team will deliver an NDA submission on schedule in 2019."

Acute attacks of migraine can differ in intensity and frequency, with many being highly disabling. More than 90% of migraine sufferers are unable to work or function normally during an attack. CGRP receptor antagonists represent a novel class of drug candidates for the treatment of migraine and are the first new class specific to the acute treatment of migraine in over 25 years. This unique and specific mode of action potentially offers an alternative to current agents. In addition, CGRP receptor antagonists could be appropriate for those who have contraindications to the use of triptans, such as patients with underlying cardiovascular diseases, or for those nonresponsive to current migraine therapies.

The patient demographics for Study 301 and Study 302 (**Table 3**) show that the population studied in these trials is representative of the typical migraine clinical population.

Table 3: Descriptive Summary of Pooled Study Demographics: Trials Representative of Typical Migraine Patient Population

Patient Demographics and Migraine History[1]
• Female > Male (~85% F; 15% M) • Average age = ~40 years old • Mean number of historical moderate to severe migraines = ~4.5 per month (range: 2-11 attacks per month) • Median historical duration of migraine attack = 24 hrs (range: 4 to 96 hours) • Photophobia was the most common historical MBS[2] as expected (~56% of the patients)
Other Study Demographics[1]
• Approximately 15% of subjects received concurrent prophylactic migraine medication

[1]Pooled demographics from Study 301 and Study 302 [2]From among nausea, phonophobia and photophobia

In summary, the efficacy and safety profile of rimegepant has now been consistently established across three randomized controlled trials to date: the two Phase 3 studies announced herein, and the previously reported Phase 2b study. The co-primary endpoints achieved in the Phase 3 trials are consistent with regulatory guidance from the U.S. Food and Drug Administration (FDA) and provide the basis for a planned submission of a new drug application (NDA) to the FDA in 2019. Data regarding additional secondary endpoints and time points beyond 8 hours are not yet available and have not yet been analyzed. Biohaven expects to present additional results from the two Phase 3 trials at upcoming scientific meetings throughout 2018.

Conference Call and Webcast

Biohaven will host a conference call and webcast today, March 26, 2018, at 8:30 a.m. ET (5:30 a.m. PT) to discuss top-line data from the two Phase 3 clinical trials. The call can be accessed by dialing 877-407-9120 (domestic) or 412-902-1009 (international). To access the audio webcast with slides, please visit the "Events" page in the Investors section of the Company's website at http://investors.biohavenpharma.com/events or directly at http://rebrand.ly/biohavenP3data. An archive of today's teleconference and webcast will be available on Biohaven's website for 30 days following the call.

About Migraine

Migraine is both a widespread and disabling neurological disorder. The Migraine Research Foundation ranks migraine as the world's third most prevalent illness, affecting approximately 36 million people or 1 out of 4 households in the United States. And the Global Burden of Disease Study 2015 rates migraine as the seventh highest specific cause of disability worldwide. Current treatment approaches, such as triptans, can be limited by headache recurrence within 24 hours after taking migraine medication, as well as cardiovascular contraindications and warnings.

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. The company'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 the Company's timing of the expected NDA submission for rimegepant and its potential to be an improved treatment option for the acute treatment of migraine, are forward-looking statements. The use of certain words, including "believe" and "will" and similar expressions, is 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 that topline data is based on preliminary analysis of key efficacy and safety data, and such data could change following a more comprehensive review and evaluation of more extensive data from the trials that the Company has not yet received, and these preliminary conclusions may not accurately reflect the complete results of the clinical trials, and uncertainties relating to the timing for submitting an NDA and the potential regulatory approval of rimegepant. Additional important factors to be considered in connection with forward-looking statements are described in the "Risk Factors" section of the Company's Annual Report on Form 10-K filed with the Securities and Exchange Commission on March 6, 2018 and other filings Biohaven makes with the U.S. Securities and Exchange Commission from time to time. 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.

For further information, contact Dr. Vlad Coric, the Chief Executive Officer at Vlad.Coric@biohavenpharma.com