Biohaven Reports Third Quarter 2023 Financial Results and Recent Business Developments

November 14, 2023

- Cash, cash equivalents, marketable securities and restricted cash totaled approximately $495 million on October 5, 2023, which included net proceeds of $242 million from completed public offering on October 5, 2023
- Completed public offering of 11,761,363 Biohaven Ltd. common shares, which included the exercise in full of the underwriters' option to purchase additional shares, at a price to the public of $22.00 per share
- Announced important updates on the Company's immunology and extracellular protein degradation platform:
    - Immunoglobulin G (IgG) degrader BHV-1300 demonstrated IgG reductions of greater than 90% from baseline with repeat dosing in non-human primates
    - BHV-1300 offers a mechanism of action that is differentiated from neonatal Fc receptor (FcRn) targeting agents with the potential for a faster onset of action, deeper reductions in IgG, no mechanistic effects on albumin or cholesterol, self-administered subcutaneous dosing, and ability to dose in conjunction with Fc-containing biologic therapeutic agents
    - Data support Biohaven's strategy of advancing BHV-1300 in combination with standard-of-care treatments for rheumatoid arthritis
    - Investigational New Drug (IND) submission for BHV-1300 remains on track for 2023 with Phase 1 initiation anticipated shortly thereafter
    - BHV-1300 provides roadmap for accelerated development of future assets from Biohaven's targeted extracellular protein degradation platform; several targets using second and third generation technologies expected to yield sustainable pipeline with potential to add significant value across rare and common diseases
    - IND submission for IgA degrader BHV-1400 for IgA nephropathy and a third undisclosed extracellular protein degrader target IND submission expected in 2024
- Announced positive data from electroencephalogram (EEG) biomarker study of BHV-7000 confirming central nervous system (CNS) activity, and other important updates including:
    - Dose-dependent and time-dependent effects on EEG across all brain regions and spectral frequencies
    - Successfully completed development of extended-release (ER) formulation of BHV-7000 to enable once-daily dosing in clinical trials; established relative bioavailability of the ER to standard release formulation
    - Differentiated safety profile from Phase 1 SAD/MAD study showing favorable CNS adverse event profile compared to other ASMs
    - Phase 3 study in focal epilepsy on track to commence in 2023 and mood disorder study to begin shortly thereafter
- Announced continued progress with taldefgrobep alfa, the Company's anti-myostatin adnectin program
    - Presented preclinical data demonstrating taldefgrobep alfa reduces fat and improves lean mass at ObesityWeek®
    - Completed enrollment in pivotal Phase 3 study of taldefgrobep alfa for the treatment of spinal muscular atrophy (SMA)
    - Taldefgrobep granted orphan drug designation (ODD) for SMA by the European Commission; taldefgrobep previously received FDA Fast-Track and ODD
- Announced successful completion of the SAD portion of the Phase 1 trial for BHV-8000, a brain penetrant TYK2/JAK1 inhibitor, and initiation of the MAD cohort to enable a Phase 2/3 clinical trial in 2024
- European Medicines Agency (EMA) informed the Company that its Marketing Authorization Application (MAA) for troriluzole (Dazluma) in the treatment of spinocerebellar ataxia has been validated and is now under review by EMA's Committee for Medicinal Products for Human Use (CHMP)

NEW HAVEN, Conn., Nov. 14, 2023 /PRNewswire/ -- Biohaven Ltd. (NYSE: BHVN) (Biohaven or the Company), 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 reported financial results for the third quarter ended September 30, 2023, and provided a review of recent accomplishments and anticipated upcoming milestones.

Vlad Coric, M.D., Chairman and Chief Executive Officer of Biohaven, commented, "Our team at Biohaven continued to make tremendous progress this past quarter in advancing multiple development programs and innovating across our entire pipeline. Most recently, we shared important data from our molecular degrader of extracellular proteins (MoDE™) program and demonstrated the potential for this technology in the field of immunology where targeting pathologic antibodies has been shown to have important therapeutic potential. We have demonstrated that BHV-1300, our lead IgG degrader, has great potential with a differentiated profile including: rapid onset of action, deeper reductions in IgG levels compared to FcRN targeting agents, lack of anticipated mechanistic effects on albumin or cholesterol, potential for self-administered subcutaneous dosing, and the compatibility to dose in combination with standard of care biologic therapeutic agents. BHV-1300 offers a highly competitive mechanism of action and potential for favorable differentiation from FcRn targeting agents. In addition, our technology can be readily modified to expand therapeutic targets past IgG to include other immunoglobulins (IgA, IgM, IgE, etc) and antigen specific autoantibodies. With multiple opportunities to address rare and common diseases alike, we continue to be impressed by the expansive potential of our degrader program.

Beyond our extracellular degraders, we also shared key data from ongoing studies with BHV-7000, our highly selective Kv7.2/7.3 activator for epilepsy, mood disorders and pain. Consistent with results observed in clinical and non-clinical studies, EEG data reported last month confirmed CNS target engagement without showing waveform increases in frequencies typically associated with adverse events commonly associated with existing ASMs, such as somnolence and other CNS effects; we expect to present additional details and analyses from this EEG study at an upcoming scientific meeting soon. With target engagement now confirmed in EEG biomarker studies, a favorable safety profile demonstrated in Phase 1 studies, and development of a once-daily formulation of BHV-7000 complete, we look forward to initiating our Phase 3 program in focal epilepsy, which we expect to commence before the end of 2023 -- a program poised to offer paradigm shifting potential in preventing seizures without burdensome CNS side effects. Separately, we completed enrollment in RESILIENT, our Phase 3 pivotal study of taldefgrobep alfa in SMA in record time, thanks to our partnership with the global SMA community and the remarkably efficient clinical trial expertise that we have in-house at Biohaven."

Dr. Coric continued, "With the closing of our recent public offering and achievement of several important milestones across our programs, we are in a strong position with access to approximately $495 million in capital following our public offering this month to continue to invest in accelerating and executing on our innovative pipeline. Our unwavering patient focus and methodical scientific approach drives every layer of our decision making, and we will progress and phase programs to balance our commitment with prudent resource allocation."

Third Quarter 2023 and Recent Business Highlights

- **Reported on continued progress with first-in-class bispecific IgG degrader, BHV-1300, as compound advances to IND submission by the end of 2023** - In September 2023, the Company announced preliminary analyses and positive pharmacodynamic data from a preclinical study evaluating BHV-1300 in cynomolgus monkeys. Repeat dosing of BHV-1300 produced dose-dependent reductions of over 90% in IgG levels from baseline, suggesting the potential for achieving greater efficacy with finely calibrated, deeper IgG reductions as compared with existing standard of care FcRn targeting treatments. The Company remains on track to submit an IND application for BHV-1300 in 2023; the Company also expects to submit an IND application for its first-in-class bispecific IgA degrader BHV-1400 in the second half of 2024.
- **Public offering -** On October 5, 2023, the Company closed its previously announced underwritten public offering of 11,761,363 of its common shares, which included the full exercise of the underwriters' option to purchase 1,534,090 additional shares, at the public offering price of $22.00 per share. The net proceeds raised in the offering, after deducting underwriting discounts and estimated expenses of the offering payable by the Company, were approximately $242.4 million. As of November 10, 2023, we had 80,233,656 common shares outstanding.
- **Announced preliminary analyses and positive biomarker data from Biohaven's exploratory Phase 1 EEG biomarker study and completed once-daily formulation development** - In September 2023, the Company announced preliminary analyses and positive biomarker data from its Phase 1 EEG biomarker study confirming CNS activity of BHV-7000 at projected therapeutic concentrations and drug concentration-dependent (i.e., dose-dependent and time-dependent) changes in EEG spectral power consistent with EEG effects observed with other ASMs approved for the treatment of epilepsy. BHV-7000 was well tolerated in the exploratory EEG study and the safety profile was consistent with the previously reported safety data from the Phase 1 SAD/MAD trial completed to date in healthy volunteers. The Company expects to share additional results from this EEG study at the upcoming American Epilepsy Society (AES) medical meeting in December.
- **Successfully dosed three cohorts with single ascending doses of oral, brain penetrant, dual TYK2/JAK1 agent, BHV-8000 -** In July 2023, the Company announced that it successfully dosed three cohorts in the SAD portion of an ongoing SAD/MAD Phase 1 study evaluating brain penetrant TYK2/JAK1 agent, BHV-8000 in healthy volunteers. The ongoing Phase 1 study is designed to evaluate the safety, tolerability, pharmacokinetics and pharmacodynamics of single and multiple ascending doses of BHV-8000 in healthy volunteers. Based on the preliminary data available, projected therapeutic concentrations of BHV-8000 were achieved, and BHV-8000 was well tolerated with only mild adverse events reported. These data provide support for further development of BHV-8000, and the Company anticipates beginning a Phase 2/3 clinical trial with BHV-8000 in Parkinson's disease and potentially other neuroinflammatory diseases in 2024.
- **Presented preclinical data demonstrating taldefgrobep alfa reduces fat and improves lean mass at ObesityWeek® -** In October 2023, the Company announced the presentation of preclinical data demonstrating the ability of taldefgrobep alfa to significantly reduce fat mass while increasing lean mass in an obese mouse model at The Obesity Society's annual ObesityWeek conference. Separately, the Company reported data supporting the correlation between change in waist circumference and change in total body weight among adults living with overweight and obesity treated with approved anti-obesity medications.
- **Completed enrollment in pivotal Phase 3 study of taldefgrobep alfa in SMA -** In September 2023, the Company announced that it had completed enrollment in RESILIENT, a pivotal Phase 3 study designed to evaluate the efficacy and

safety of taldefgrobep as adjunctive therapy to enhance muscle mass and function in SMA patients treated with standard-of-care treatments. Taldefgrobep is the only myostatin inhibitor in clinical development that targets both myostatin and activin A signaling, two key regulators of muscle mass. In July 2023, the Company announced that taldefgrobep received ODD from the European Commission for the treatment of SMA. Taldefgrobep previously received Fast-Track and ODD from the FDA.

Expected Upcoming Milestones:

Biohaven is progressing its product candidates through clinical programs in a number of common and rare disorders. The Company expects to reach significant pipeline milestones in the coming periods. Biohaven expects to:

- **Announce additional Phase 1 EEG study results for Kv7 activator BHV-7000 at the American Epilepsy Society Annual Meeting in December:** The Company expects to present complete results from its EEG study with BHV-7000 in healthy volunteers by the end of the year.
- **Initiate Phase 3 programs with BHV-7000 in the second half of 2023 and first half of 2024:** Biohaven expects to initiate pivotal trials in patients with focal epilepsy by the end of 2023 and bipolar disorder in the first half of 2024.
- **Submit IND with BHV-1300, the Company's lead extracellular degrader:** The Company expects to advance our IND for the lead IgG degrader BHV-1300 by year-end 2023.
- **Submit IND with selective Gd-IgA1 degrader BHV-1400:** The Company expects to submit an IND with Gd-IgA1 degrader BHV-1400, indicated for IgA nephropathy, in the second half of 2024.
- **Initiate Phase 2/3 study with brain penetrant, dual TYK2/JAK1 inhibitor BHV-8000 in Parkinson's disease:** The Company commenced Phase 1 studies with BHV-8000, an oral, brain-penetrant, dual TYK2/JAK1 inhibitor for neuroinflammatory disorders, in the first half of 2023 and expects to initiate a Phase 2/3 study in Parkinson's disease in 2024.
- **Submit IND with TRPM3 antagonist BHV-2100:** The Company expects to submit an IND with BHV-2100, a selective TRPM3 antagonist in the Company's ion channel platform, indicated for pain disorders, including migraine, by year-end 2023.
- **Taldefgrobep alfa program updates**: The Company expects to initiate a Phase 2 trial in metabolic disorders in 2024.
- **Complete enrollment in Phase 3 studies of troriluzole in OCD in 2024:** Two Phase 3 randomized, double-blind, placebo-controlled studies of troriluzole in OCD are expected to enroll up to 700 patients (in each trial) across nearly 200 global study sites. The Company anticipates completing enrollment in the Phase 3 trials in 2024.
- **Continue advancements across multiple neuroscience and immunoscience indications:** The Company's preclinical pipeline includes a platform of bispecific degraders of extracellular proteins directed against IgG, IgA and other targets, TRPM3 and Kv7 family of ion channel modulators, and other undisclosed targets.

Capital Position:

Cash, cash equivalents, marketable securities and restricted cash totaled approximately $495 million as of October 5, 2023, which included net proceeds of $242 million from completed public offering on October 5, 2023.

Third Quarter 2023 Financial Highlights:

Research and Development (R&D) Expenses: R&D expenses, including non-cash share-based compensation costs, were $95.5 million for the three months ended September 30, 2023, compared to $52.8 million for the three months ended September 30, 2022. The increase of $42.7 million was primarily due to increases in direct program spend for additional and advancing clinical trials, including late Phase 2/3 studies, and preclinical research programs in 2023, as compared to the same period in the prior year. Non-cash share-based compensation expense was $2.2 million for the three months ended September 30, 2023, a decrease of $7.5 million as compared to the same period in 2022. Non-cash share-based compensation expense was higher in the third quarter of 2022 primarily because expense allocated from Biohaven Pharmaceutical Holding Company Ltd.'s (the Former Parent[1]) equity plan, prior to the spin-off, was based on equity awards with higher grant date fair values, which was partially offset by increased personnel costs related to increased headcount in 2023.

General and Administrative (G&A) Expenses: General and administrative expenses were $15.0 million for the three months ended September 30, 2023, compared to $14.8 million for the three months ended September 30, 2022. The increase of $0.2 million was increased personnel costs in the third quarter of 2023 due to a majority of the personnel costs in three months ended September 30, 2022 being allocated to the Former Parent, offset by decreased non-cash share-based compensation costs. Non-cash share-based compensation expense was $2.3 million for the three months ended September 30, 2023, a decrease of $5.0 million as compared to the same period in 2022. Non-cash share-based compensation expense was higher in the third quarter of 2022 primarily because expense allocated from the Former Parent equity plan, prior to the spin-off, was based on equity awards with higher grant date fair values.

Other Income (Expense), Net: Other income (expense), net was a net income of $4.7 million for the three months ended September 30, 2023. The Company did not record any other income (expense) for the three months ended September 30, 2022. The increase of $4.7 million was primarily due to an increase in net investment income of $3.8 million and an increase of $1.2 million in other income related to our transition services provided to the Former Parent, which is largely non-recurring.

Net Loss: Biohaven reported a net loss for the three months ended September 30, 2023, of $102.6 million, or $1.50 per share, compared to $68.9 million, or $1.75 per share, for the same period in 2022. Non-GAAP adjusted net loss for the three months ended September 30, 2023 was $98.1 million, or $1.44 per share, compared to $49.2 million, or $1.25 per share for the same period in 2022. These non-GAAP adjusted net loss and non-GAAP adjusted net loss per share measures, more fully described below under "Non-GAAP Financial Measures," exclude non-cash share-based

compensation charges. A reconciliation of the GAAP financial results to non-GAAP financial results is included in the tables below. For periods prior to the Spin-Off, net loss per share and non-GAAP adjusted net loss per share were calculated based on the 39,375,944 common shares of Biohaven distributed to the Former Parent shareholders at the time of the distribution, including common shares issued in connection with the Former Parent share options that were settled on October 3, 2022 and common shares issued in connection with the Former Parent restricted share units that vested on October 3, 2022. The same number of shares is being utilized for the calculation of basic and diluted earnings per share for all periods presented prior to the Spin-Off.

Non-GAAP Financial Measures

This press release includes financial results prepared in accordance with accounting principles generally accepted in the United States (GAAP), and also certain non-GAAP financial measures. In particular, Biohaven has provided non-GAAP adjusted net loss and adjusted net loss per share, which are adjusted to exclude non-cash share-based compensation, which is substantially dependent on changes in the market price of common shares, and transaction-related cost incurred relating to the company's spin-off from Biohaven Pharmaceutical Holding Company Ltd., which are limited to a specific period of time and related to Biohaven Ltd. being established as a standalone public company. Non-GAAP financial measures are not an alternative for financial measures prepared in accordance with GAAP. However, Biohaven believes the presentation of non-GAAP adjusted net loss and adjusted net loss per share, when viewed in conjunction with GAAP results, provides investors with a more meaningful understanding of ongoing operating performance and can assist investors in comparing Biohaven's performance between periods.

In addition, these non-GAAP financial measures are among those indicators Biohaven uses as a basis for evaluating performance, and planning and forecasting future periods. These non-GAAP financial measures are not intended to be considered in isolation or as a substitute for GAAP financial measures. A reconciliation between these non-GAAP measures and the most directly comparable GAAP measures is provided later in this news release.

About Biohaven

Biohaven is 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. 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 and schizophrenia. The company is advancing a pipeline of therapies for diseases, many of which have limited 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 metabolic disorders, and brain-penetrant TYK2/JAK1 inhibition for neuroinflammatory 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 degradation 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 "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 FDA; 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 news 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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[1] On May 9, 2022, the Board of Directors of the Former Parent approved and directed the Former Parent's management to effect the spin-off (the "Spin-Off") of the Kv7 ion channel activators, glutamate modulation and myostatin inhibition platforms, preclinical product candidates, and certain corporate infrastructure then owned by the Former Parent in connection with the sale of the Former Parent to Pfizer. On October 3, 2022, the purchase of the Former Parent by Pfizer was consummated and the Former Parent completed the distribution to holders of its common shares of all of the outstanding common shares of Biohaven Ltd.

BIOHAVEN LTD.
CONDENSED CONSOLIDATED STATEMENTS OF OPERATIONS
(Amounts in thousands, except share and per share amounts)
(Unaudited)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2023	**2022**	**2023**	**2022**
Operating expenses:				
Research and development	$ 95,517	$ 52,845	$ 238,468	$ 300,028
General and administrative	15,030	14,792	43,872	54,492
Total operating expenses	110,547	67,637	282,340	354,520
Loss from operations	(110,547)	(67,637)	(282,340)	(354,520)

Other income (expense), net	4,686	—	18,757	(71)
Loss before (benefit) provision for income taxes	(105,861)	(67,637)	(263,583)	(354,591)
(Benefit) provision for income taxes	(3,287)	1,216	(171)	14,581
Net loss	$ (102,574)	$ (68,853)	$ (263,412)	$ (369,172)
Net loss per share — basic and diluted	$ (1.50)	$ (1.75)	$ (3.86)	$ (9.38)
Weighted average common shares outstanding— basic and diluted	68,320,125	39,375,944	68,258,757	39,375,944

BIOHAVEN LTD.
CONDENSED CONSOLIDATED BALANCE SHEETS
(Amounts in thousands)

	September 30, 2023 (Unaudited)	December 31, 2022
Assets		
Current assets:		
Cash and cash equivalents	$ 111,697	$ 204,877
Marketable securities	128,899	260,464
Prepaid expenses	33,936	20,945
Income tax receivable	13,073	46,139
Restricted cash held on behalf of Former Parent	28	35,212
Other current assets	22,937	19,331
Total current assets	310,570	586,968
Property and equipment, net	17,669	17,512
Intangible assets	18,400	18,400
Goodwill	1,390	1,390
Other non-current assets	34,707	37,513
Total assets	$ 382,736	$ 661,783
Liabilities and Equity		
Current liabilities:		
Accounts payable	$ 9,515	$ 10,703
Due to Former Parent	28	35,212
Accrued expenses and other current liabilities	52,634	44,106
Total current liabilities	62,177	90,021
Long-term operating lease liability	28,286	30,581
Other non-current liabilities	2,267	2,410
Total liabilities	92,730	123,012
Shareholders' Equity:		
Preferred shares, no par value; 10,000,000 shares authorized, no shares issued and outstanding as of September 30, 2023 and December 31, 2022	—	—
Common shares, no par value; 200,000,000 shares authorized as of September 30, 2023 and December 31, 2022; 68,364,143 and 68,190,479 shares issued and outstanding as of September 30, 2023 and December 31, 2022, respectively	618,761	615,742
Additional paid-in capital	25,623	13,869
Accumulated deficit	(354,536)	(91,124)
Accumulated other comprehensive income	158	284
Total shareholders' equity	290,006	538,771
Total liabilities and shareholders' equity	$ 382,736	$ 661,783

BIOHAVEN LTD.
RECONCILIATION OF GAAP TO NON-GAAP FINANCIAL MEASURES
(Amounts in thousands, except share and per share amounts)
(Unaudited)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2023	2022	2023	2022
Reconciliation of GAAP to Non-GAAP adjusted net loss:				
GAAP net loss	$ (102,574)	$ (68,853)	$ (263,412)	$ (369,172)

Add: non-cash share-based compensation expense	4,456	16,997	12,916	77,927
Add: Transaction-related costs	—	2,641	—	5,863
Non-GAAP adjusted net loss	$ (98,118)	$ (49,215)	$ (250,496)	$ (285,382)

Reconciliation of GAAP to Non-GAAP adjusted net loss per share — basic and diluted:

GAAP net loss per share — basic and diluted	$ (1.50)	$ (1.75)	$ (3.86)	$ (9.38)
Add: non-cash share-based compensation expense	0.07	0.43	0.19	1.98
Add: Transaction-related costs	—	0.07	—	0.15
Non-GAAP adjusted net loss per share — basic and diluted	$ (1.44)	$ (1.25)	$ (3.67)	$ (7.25)

MoDEs is a trademark of Biohaven Therapeutics Ltd.

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