Biohaven Reports First Quarter 2023 Financial Results and Reports Recent Business Developments

May 12, 2023

- Acquired exclusive license for oral, brain-penetrant, dual TYK2/JAK1 inhibitor for immune-mediated brain disorders in March 2023 covering global rights (excluding China)
- Appointed Nick Kozauer, M.D. as SVP of Clinical Development and Regulatory Strategy following his tenure as Director of the Division of Neurology 2 in the Office of New Drugs of the U.S. Food and Drug Administration
- Taldefgrobep alfa granted Fast Track Designation in SMA
- Driving strong and consistent progress across six robust drug development platforms in 2023:
    - in Kv7 activation, targeting Phase 2/3 study start in focal epilepsy and bipolar disorder in the second half of 2023;
    - Phase 1 study initiation planned with potentially first-in-class TYK2/JAK1 brain-penetrant inhibitor in immune-mediated brain disorders;
    - three Phase 3 studies gaining momentum with regulatory engagement and enrollment completion expected in programs spanning glutamate modulation in SCA and OCD, respectively, and enrollment ongoing in myostatin inhibition program in SMA;
    - three IND submissions anticipated in 2023 spanning Kv7 activation, TRPM3 antagonism, and IgG degradation offer potential to drive long-term value creation beyond 2023

NEW HAVEN, Conn., May 12, 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 for people with debilitating neurological and neuropsychiatric diseases, including ultra-rare disorders, today reported financial results for the first quarter ended March 31, 2023, and provided a review of recent accomplishments and anticipated upcoming milestones.

Vlad Coric, M.D., Chairman and Chief Executive Officer of Biohaven, commented, "We have set forth ambitious goals for the balance of 2023, as we continue advancing what we believe is one of the most innovative and exciting neuroscience platform in development. We were energized by the preliminary safety, tolerability and pharmacokinetic data we reported from our SAD/MAD Phase 1 study of Kv7.2/Kv7.3 activator BHV-7000 earlier in the quarter, supported by the preponderance of preclinical efficacy data generated to date with BHV-7000 demonstrating potent anti-seizure efficacy, concentration-dependent hyperpolarization of the resting membrane potential, meaningful increases in action potential threshold, and encouragingly low $GABA_A$ activity. We also look forward to unlocking Kv7 platform expansion opportunities across a spectrum of indications with high unmet medical need."

Dr Coric continued, "With our glutamate platform, we look to gain clarity on the regulatory path forward for SCA and expect to complete enrollment in our Phase 3 OCD program in 2023, which we believe are two critically important milestones given the dearth of approved therapies for these distinct patient populations. Regarding our myostatin platform, we continue to activate sites and drive enrollment in our ongoing Phase 3 SMA study. With our recently acquired TYK2/JAK1 platform, we remain focused on starting Phase 1 studies in 2023 and exploring BHV-8000's potential to address an array of immune-mediated brain disorders. And finally, we look forward to submitting IND applications across three separate programs, including BHV-7010, a next-gen Kv7 activator targeting epilepsy and mood disorders, BHV-2100, a TRPM3 inhibitor targeting chronic pain, and BHV-1300, our IgG degrader, following the report of robust preclinical data earlier in the year. We remain as committed as ever to driving data-driven, efficient results for patients and the shareholders who support our continued work, and look forward to executing and delivering continued value in the year ahead."

First Quarter 2023 and Recent Business Highlights

- **Acquired BHV-8000, a brain-penetrant inhibitor of TYK2/JAK1, from Highlightll** - As previously reported, in March 2023, the Company acquired exclusive rights (excluding China) to a novel, oral, first-in-class, brain-penetrant, dual inhibitor of TYK2/JAK1 offering wide therapeutic index with TYK2 inhibition and high selectivity for JAK1 inhibition without the severely limiting adverse class effects of JAK2/JAK3 inhibitors. The Company expects to commence Phase 1 development in 2023, exploring its potential to address immune-mediated brain disorders.
- **Delivered oral and poster presentations demonstrating preclinical efficacy and initial Phase 1 safety and tolerability of BHV-7000 on ASENT 2023 virtual platform -** In March 2023, the Company presented data at the 2023 American Society for Experimental Neurotherapeutics Annual Meeting (ASENT 2023) demonstrating that activating Kv7.2/7.3 with BHV-7000, a structurally and pharmacologically distinct compound from ezogabine, produced concentration-

dependent hyperpolarization of the resting membrane potential, potentiated meaningful increases in action potential threshold, exhibited significantly lower $GABA_A$ activity than ezogabine, and delivered potent anti-seizure efficacy in the maximal electroshock seizure (MES) model, without negatively impacting neurobehavior. The Company also presented data from the Phase 1 SAD/MAD clinical trial, demonstrating BHV-7000 was well-tolerated at single doses up to 100 mg and multiple doses up to 40 mg per day for 15 days. Most adverse events were mild and resolved spontaneously, and there were no serious or severe adverse events or dose-limiting toxicities reported. In BHV-7000 treated subjects in the MAD study, CNS-related adverse events typically associated with other anti-seizure medications were not reported.

- **Taldefgrobep Alfa Granted Fast Track Designation and Orphan Drug Designation** - As previously reported, Taldefgrobep alfa, an anti-myostatin adnectin in development for SMA, was granted Fast Track designation and Orphan Drug designation by the U.S. Food and Drug Administration (FDA) in February 2023, and December 2022, respectively. Phase 3 studies are ongoing; the Company expects to enroll approximately 180 patients in this randomized, double-blind, placebo-controlled global trial.
- **Reported preclinical data with extracellular target degrader platform technology (MoDE™), a pan-IgG degrader**- As previously reported, in January 2023, the Company evaluated the effect of a single dose of IgG degrader, BHV-1300, in cynomolgus monkeys. The Company reported 75% reduction of IgG levels from baseline and noted the observation occurred in three days; the data in this pre-clinical study compares favorably to standard of care therapy efgartigimod, where reduction of IgG levels with efgartigimod was observed to be 50% and had taken 5-7 days. The Company expects BHV-1300 will be ready for IND submission in the second half of 2023.
- **Reported preclinical data with second MoDE in bispecific platform targeting IgA Nephropathy** - As previously reported, in January 2023, the Company reported preclinical data with a second MoDE targeting galactose deficient IgA (Gd-IgA), which is believed to play a pathogenic role in IgA Nephropathy. Specific removal of pathogenic Gd-IgA with preservation of normal IgA potentially permits disease remission without incurring an infection risk. The Company shared preliminary data demonstrating the chimeric antibody-ASGPR ligand conjugate specifically mediated endocytosis of Gd-IgA, as opposed to normal IgA, in an endocytosis assay with HepG2 cells.
- **Leadership team expanded with key appointment** - In April 2023, Biohaven announced the appointment of Nick Kozauer, M.D. as SVP of Clinical Development and Regulatory Strategy following his tenure as Director of the Division of Neurology 2 in the Office of New Drugs of the FDA. Dr. Kozauer had supervised and reviewed the approval of over 15 marketed drugs during his tenure at the FDA.

Upcoming Expected 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:

- **Initiate EEG study with BHV-7000 in the first half of 2023:** Following Phase 1 study completion, Biohaven expects to initiate pivotal trials in patients with epilepsy and patients with bipolar disorder in the second half of 2023.
- **Submit IND with BHV-7010 in epilepsy and mood disorders:** The Company expects to submit an IND with next-generation Kv7 activator BHV-7010 in epilepsy in the second half of 2023.
- **Submit IND with BHV-2100 in chronic pain:** The Company expects to submit an IND with BHV-2100, a TRPM3 antagonist in the Company's ion channel platform targeting a pain disorder in the second half of 2023.
- **Submit IND with BHV-1300:** The Company expects to submit an IND with pan-IgG degrader BHV-1300 in the second half of 2023.
- **Commence Phase 1 studies with BHV-8000:** The Company expects to commence Phase 1 studies with BHV-8000, an oral, brain-penetrant, dual TYK2/JAK1 inhibitor for immune-mediated brain disorders in 2023.
- **Complete enrollment in Phase 3 study of troriluzole in OCD in 2023:** Two Phase 3 randomized, double-blind, placebo-controlled studies are expected to enroll up to 700 patients (in each trial) across nearly 200 global study sites. The Company anticipates completing enrollment in year-end 2023.
- **Provide an update on troriluzole in SCA:** The Company intends to interact with the FDA and/or European Medicines Agency in the first half of 2023 on next steps.
- **Continue advancing Phase 3 clinical studies of taldefgrobep alfa in SMA:** The Company expects to enroll approximately 180 patients in the study through patient enrollment in up to 60 clinical sites.
- **Continue advancements across multiple neuroscience and immunoscience indications:** The Company's preclinical pipeline includes molecular degraders of extracellular proteins, CD38 targeting antibody recruiting molecules (ARMs), TRP channels, and other undisclosed targets, including those with disease-modifying potential.

Capital Position:

Cash, cash equivalents and marketable securities as of March 31, 2023 was $392.0 million, including $4.0 million of restricted cash, and excluding $61.5 million of cash payable to Biohaven Pharmaceutical Holding Company Ltd. (the "Former Parent"), compared to $467.9 million, including $2.5 million of restricted cash, and excluding $35.2 million of cash payable to the Former Parent, as of December 31, 2022[1].

First Quarter 2023 Financial Highlights:

Research and Development (R&D) Expenses: R&D expenses, including non-cash share-based compensation costs, were $63.5 million for the three months ended March 31, 2023, compared to $70.1 million for the three months ended March 31, 2022. The decrease of $6.6 million was primarily due to a decrease of $14.7 million in personnel-related costs including non-cash share-based compensation costs, and a decrease in expenses for verdiperstat, BHV-2100 and BHV-1200, partially offset by an increase in expenses for our clinical programs for Kv7 (BHV-7000 and 7010), troriluzole and BHV-2000. The decrease in personnel-related costs is due to non-cash share-based compensation expense for the first quarter of 2022 being allocated from the Former Parent equity plan based on equity awards with higher grant date fair values, which was partially offset by increased personnel costs related to an increase in headcount for our discovery operations. Non-cash share-based compensation expense was $2.2 million for the three months ended March 31, 2023, a decrease of $22.3 million as compared to the same period in 2022.

General and Administrative (G&A) Expenses: G&A expenses, including non-cash share-based compensation costs, were $14.3 million for the three months ended March 31, 2023, compared to $19.7 million for the three months ended March 31, 2022. The decrease of $5.4 million was primarily due to decreased non-cash share-based compensation costs. This was partially offset by increased personnel costs in the first quarter of 2023 compared to the same period in 2022, due to a majority of the personnel costs in the first quarter of 2022 being allocated to the Former Parent. Non-cash share-based compensation expense was $1.5 million for the three months ended March 31, 2023, a decrease of $14.1 million as compared to the same period in 2022. The decrease in non-cash share-based compensation expense is due to the first quarter of 2022 expense being allocated from the Former Parent equity plan based on equity awards with higher grant date fair values.

Other Income (Expense), Net: Other income (expense), net was a net income of $8.2 million for the three months ended March 31, 2023, compared to net expense of $4.0 thousand for the three months ended March 31, 2022. The increase of $8.2 million was primarily due to an increase in net investment income and an increase of $3.9 million in other income related to our transition services provided to our Former Parent, which is largely non-recurring.

Net Loss: Biohaven reported a net loss for the three months ended March 31, 2023, of $70.5 million, or $1.03 per share, compared to $97.0 million, or $2.46 per share, for the same period in 2022. Non-GAAP adjusted net loss for the three months ended March 31, 2023 was $66.7 million, or $0.98 per share, compared to $56.9 million, or $1.45 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 Biohaven's spin-off from the Former Parent on October 3, 2022 (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. 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 for people with debilitating neurological and neuropsychiatric 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 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 extracellular target degrader 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 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.

[1] On May 9, 2022, the Board of Directors of the Former Parent approved and directed Former Parent's management to effect 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 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 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 March 31,	
	2023	**2022**
Operating expenses:		
Research and development	$ 63,461	$ 70,096
General and administrative	14,321	19,677
Total operating expenses	77,782	89,773
Loss from operations	(77,782)	(89,773)
Other income (expense):		
Other income (expense), net	8,229	(4)
Total other income (expense), net	8,229	(4)
Loss before provision for income taxes	(69,553)	(89,777)
Provision for income taxes	939	7,255
Net loss	$ (70,492)	$ (97,032)
Net loss per share — basic and diluted	$ (1.03)	$ (2.46)
Weighted average common shares outstanding— basic and diluted	68,206,879	39,375,944

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

	March 31, 2023 (Unaudited)	**December 31, 2022**
Assets		
Current assets:		
Cash and cash equivalents	$ 125,031	$ 204,877
Marketable securities	262,998	260,464
Prepaid expenses	26,400	20,945
Income tax receivable	43,830	46,139
Restricted cash held on behalf of Former Parent	61,548	35,212
Other current assets	24,669	19,331
Total current assets	544,476	586,968
Property and equipment, net	17,494	17,512
Intangible assets	18,400	18,400
Goodwill	1,390	1,390
Other non-current assets	36,761	37,513
Total assets	$ 618,521	$ 661,783
Liabilities and Equity		
Current liabilities:		
Accounts payable	$ 14,456	$ 10,703
Due to Former Parent	61,548	35,212
Accrued expenses and other current liabilities	38,002	44,106
Total current liabilities	114,006	90,021
Long-term operating lease liability	29,760	30,581
Other non-current liabilities	2,497	2,410
Total liabilities	146,263	123,012
Shareholders' Equity:		

Preferred shares, no par value; 10,000,000 shares authorized, no shares issued and outstanding as of March 31, 2023 and December 31, 2022	—	—
Common shares, no par value; 200,000,000 shares authorized as of March 31, 2023 and December 31, 2022; 68,212,479 and 68,190,479 shares issued and outstanding as of March 31, 2023 and December 31, 2022, respectively	616,246	615,742
Additional paid-in capital	17,462	13,869
Accumulated deficit	(161,616)	(91,124)
Accumulated other comprehensive income	166	284
Total shareholders' equity	472,258	538,771
Total liabilities and shareholders' equity	$ 618,521	$ 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 March 31,	
	2023	2022
Reconciliation of GAAP to Non-GAAP adjusted net loss:		
GAAP net loss	$ (70,492)	$ (97,032)
Add: non-cash share-based compensation expense	3,765	40,120
Non-GAAP adjusted net loss	$ (66,727)	$ (56,912)
Reconciliation of GAAP to Non-GAAP adjusted net loss per share — basic and diluted:		
GAAP net loss per share — basic and diluted	$ (1.03)	$ (2.46)
Add: non-cash share-based compensation expense	0.05	1.02
Non-GAAP adjusted net loss per share — basic and diluted	$ (0.98)	$ (1.45)

MoDEs is a trademark of Biohaven Therapeutics Ltd.

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