Biohaven Reports Third Quarter 2024 Financial Results and Recent Business Developments

November 12, 2024

- Cash, cash equivalents, marketable securities and restricted cash as of October 2, 2024 totaled approximately $642 million
- Achieved positive topline trial results from pivotal trial with troriluzole in spinocerebellar ataxia (SCA)
    - Troriluzole 200 mg QD dosed orally in patients with SCA met the study's primary endpoint on the change from baseline on the modified functional Scale for the Assessment and Rating of Ataxia (f-SARA) at 3 years in all study population genotypes
    - Statistically significant superiority achieved on 9 consecutive, prespecified primary and secondary endpoints
    - Both the study protocol and statistical analysis plan were submitted to, and reviewed by, the U.S. Food and Drug Administration (FDA) prior to topline data analysis
    - Study designed in discussion with the FDA and utilized Phase 3 data and an external control of matched, untreated SCA subjects from the U.S. Clinical Research Consortium for the Study of Cerebellar Ataxia (CRC-SCA) in accordance with the FDA's Guidance on Real-World Evidence (RWE) of effectiveness
    - CRC-SCA external control included contemporaneous natural history data gathered from 2010-2024
    - Planned New Drug Application (NDA) re-submission in 4Q 2024
    - Completed clarification meeting with CHMP Rapporteurs in 4Q 2024 and MAA documents are being updated to include the new positive BHV4157-206-RWE study data with broader indication to include all SCA genotypes
- Taldefgrobep alfa, a myostatin-inhibitor, Phase 3 topline data in spinal muscular atrophy (SMA) in 4Q 2024 and Phase 2 trial protocol in obesity expected in 4Q 2024
- Advancing extracellular Molecular Degrader of Extracellular Protein (MoDE) programs
    - 3 additional investigational agents expected to enter Phase 1 studies in the next quarter
    - Anticipate Phase 1 update for BHV-1300, including subcutaneous formulation, before year-end
- Broad progress with TRPM3 antagonist
    - Initiated pivotal Phase 2 trial evaluating BHV-2100, a TRPM3 antagonist, in the acute treatment of migraine
    - Initiated separate proof of concept study with BHV-2100 in neuropathic pain
- Patient enrollment continues across 5 Phase 2/3 trials with Kv7 activator, BHV-7000, in epilepsy and mood disorders (bipolar and major depressive disorder (MDD)) with potential for multiple data readouts in 2025
- Continued progress with antibody drug conjugate (ADC) portfolio
    - BHV-1510 currently dosing cancer patients in Phase 1/2 study, advancing towards combination dosing of BHV-1510 with Libtayo® in 4Q 2024

NEW HAVEN, Conn., Nov. 12, 2024 /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, 2024, and provided a review of recent accomplishments and anticipated upcoming developments.

Vlad Coric, M.D., Chairman and Chief Executive Officer of Biohaven, commented, "The team at Biohaven continues to advance multiple late and early stage assets that have the potential to change the current standard of care treatment paradigm across a number of diseases. Last quarter we announced positive topline results in our RWE trial assessing troriluzole for the treatment of spinocerebellar ataxia. Troriluzole (200 mg dosed orally) met the study's primary endpoint on the change from baseline in the f-SARA at 3 years in all study population genotypes, showed statistically significant superiority after both 1 and 2 years of treatment, and achieved statistically significant superiority on 9 consecutive, prespecified primary and secondary endpoints. This was truly a watershed moment for the Company, given the implications of potentially bending the arc of one of the most intractable neurological diseases with no currently approved treatment options. SCA robs patients of their ability to speak, walk, and take care of themselves, and often shortens lifespans and has unfortunate intergenerational implications. We were encouraged by the robust dataset gathered to date and look forward to submitting an NDA to the FDA."

Dr. Coric continued, "We continue to execute across our broad pipeline including the recent initiation of our Phase 2 trial with BHV-2100 in acute migraine. Migraine burden and disability remain high despite advances in treatment; we believe our TRPM3 antagonistic approach has the potential to be a highly-effective, non-sedating, non-opioid treatment for pain and migraine. In the final months of 2024, we expect to report on a number of

updates including SMA topline data and data across our MoDE™ platform including a SAD/MAD update and multiple INDs spanning β1AR for the potential treatment of dilated cardiomyopathy, galactose deficient IgA for IgA nephropathy and a further optimized IgG degrader for use in rare diseases."

Third Quarter 2024 and Recent Business Highlights

- ***Achieved positive topline results in pivotal study of troriluzole in SCA -*** In September 2024, the Company announced positive topline results from pivotal Study BHV4157-206-RWE demonstrating the efficacy of troriluzole on the mean change from baseline in the f-SARA after 3 years of treatment. The study achieved the primary endpoint and showed statistically significant improvements on the f-SARA at years 1 and 2. Additionally, troriluzole achieved statistically significant superiority on 9 consecutive, prespecified primary and secondary endpoints. SCA patients treated with troriluzole showed a 50-70% slowing of disease progression, representing 1.5-2.2 years delay in disease progression over the 3-year study period. The Company intends to submit an NDA to the FDA for troriluzole in the treatment of all SCA genotypes in 4Q 2024. The development program for troriluzole has been granted orphan and fast track designations, and is eligible for priority review. European Medicines Agency marketing authorization remains under review and Biohaven completed a clarification meeting with CHMP Rapporteurs in 4Q 2024. MAA documents are being updated to include the new positive BHV4157-206-RWE study data with broader indication to include all SCA genotypes.

- ***Initiated Phase 2 trial evaluating BHV-2100 in the acute treatment of migraine -*** In September 2024, the Company initiated a Phase 2 study of an orally administered TRPM3 antagonist, BHV-2100, in the acute treatment of migraine. The study is a randomized, double-blind, placebo-controlled trial assessing the efficacy and safety of two doses (75 mg and 150 mg) of BHV-2100 in the acute treatment of migraine. The trial is designed to support registration with FDA-accepted co-primary endpoints of pain freedom and freedom from most bothersome symptom at 2 hours and is expected to enroll approximately 575 patients across 60 sites in the United States.

- ***Public offering*** - On October 2, 2024, the Company closed its previously announced underwritten public offering of 6,052,631 of its common shares, which included the full exercise of the underwriters' option to purchase additional shares, at the public offering price of $47.50 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 $269.9M. As of November 8, 2024, we had 101,122,246 common shares outstanding.

Expected Upcoming Milestones:

We believe Biohaven is well positioned to achieve significant milestones in 2024 and 2025 across numerous programs:

Selective Kv7 Activator:

- Continue 5 ongoing Phase 2/3 trials with BHV-7000 in focal epilepsy, idiopathic generalized epilepsy, MDD, and bipolar disorder

Troriluzole:

- NDA submission to FDA on track for 4Q 2024, following release of pivotal topline results in SCA in September 2024
- Two Phase 3 trials with troriluzole in OCD; expect to conduct interim analysis of the second Phase 3 OCD trial in 4Q 2024 and report topline data from first Phase 3 OCD trial in 1H 2025

Taldefgrobep alfa:

- Report topline data from Phase 3 trial with taldefgrobep in SMA in 4Q 2024
- Initiate Phase 2 trial with taldefgrobep in obesity in 4Q 2024 or early 2025

First-in-class TRPM3 Antagonist:

- Continue advancing enrollment in Phase 2 trial with BHV-2100 in acute migraine and neuropathic pain (laser-evoked potential experimental pain paradigm)

TYK2/JAK1 Inhibitor:

- Complete SAD/MAD studies with BHV-8000 and advance to Phase 2 in the coming months

MoDE™ Platform

- Submit a total of 4 INDs in 2024
- Continue to advance Phase 1 SAD and MAD studies with subcutaneous BHV-1300, with a further study update in 4Q 2024

Next Generation ADC Platform:

- Advance Phase 1 Trop-2 directed program BHV-1510 in multiple tumor types

Capital Position:

Cash, cash equivalents, marketable securities and restricted cash as of October 2, 2024 totaled approximately $642 million, which includes net proceeds of $269.9 million from the public offering of 6,052,631 common shares completed on October 2, 2024.

Third Quarter 2024 Financial Highlights:

Research and Development (R&D) Expenses: R&D expenses, including non-cash share-based compensation costs, were $157.6 million for the three months ended September 30, 2024, compared to $95.5 million for the three months ended September 30, 2023. The increase of $62.1 million was due to additional and advancing clinical trials, including late Phase 3 and Phase 2/3 studies, and preclinical research programs in 2024, as compared to the same period in the prior year. Non-cash share-based compensation expense was $7.2 million for the three months ended September 30, 2024, an increase of $5.0 million as compared to the same period in 2023. Non-cash share-based compensation expense was higher in the third quarter of 2024, primarily due to our annual equity incentive awards granted in the fourth quarter of 2023 and the first quarter of 2024.

General and Administrative (G&A) Expenses: G&A expenses were $20.6 million for the three months ended September 30, 2024, compared to $15.0 million for the three months ended September 30, 2023. The increase of $5.5 million was partly due to increased non-cash share-based compensation expense, which was $5.0 million for the three months ended September 30, 2024, an increase of $2.7 million as compared to the same period in 2023. Non-cash share-based compensation expense was higher in the third quarter of 2024 primarily due to our annual equity incentive awards granted in the fourth quarter of 2023 and the first quarter of 2024.

Other Income, Net: Other income, net was $17.8 million for the three months ended September 30, 2024, compared to other income, net of $4.7 million for the three months ended September 30, 2023. The increase of $13.1 million was primarily due to non-cash changes in the fair value of our derivative liabilities recorded in connection with the amendment to our Membership Interest Purchase Agreement with Knopp Biosciences LLC in May 2024 (the Knopp Amendment), as well as increased investment income, partially offset by changes in the fair value of our forward contract liability recorded in connection with the Knopp Amendment.

Net Loss: Biohaven reported a net loss for the three months ended September 30, 2024 of $160.3 million, or $1.70 per share, compared to $102.6 million, or $1.50 per share, for the same period in 2023. Non-GAAP adjusted net loss for the three months ended September 30, 2024 was $164.1 million, or $1.74 per share, compared to $98.1 million, or $1.44 per share for the same period in 2023. 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 and losses from the change in fair value of derivatives. A reconciliation of the GAAP financial results to non-GAAP financial results is included in the tables below.

Non-GAAP Financial Measures
This press release includes financial results prepared in accordance with accounting principles generally accepted in the United States (GAAP), and 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 changes in the fair value of derivative liabilities, which do not correlate to actual cash payment obligations in the relevant periods. 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 biopharmaceutical company focused on the discovery, development, and commercialization of life-changing treatments in key therapeutic areas, including immunology, neuroscience, and oncology. The company is advancing its innovative portfolio of therapeutics, leveraging its proven drug development experience and multiple proprietary drug development platforms. Biohaven's extensive clinical and preclinical programs include Kv7 ion channel modulation for epilepsy and mood disorders; extracellular protein degradation for immunological diseases; TRPM3 antagonism for migraine and neuropathic pain; TYK2/JAK1 inhibition for neuroinflammatory disorders; glutamate modulation for OCD and SCA; myostatin inhibition for neuromuscular and metabolic diseases, including SMA and obesity; antibody recruiting bispecific molecules and antibody drug conjugates for cancer.

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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CONSOLIDATED STATEMENTS OF OPERATIONS

(Amounts in thousands, except share and per share amounts)

(Unaudited)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2024	**2023**	**2024**	**2023**
Operating expenses:				
Research and development	$ 157,607	$ 95,517	$ 628,398	$ 238,468
General and administrative	20,561	15,030	66,782	43,872
Total operating expenses	178,168	110,547	695,180	282,340
Loss from operations	(178,168)	(110,547)	(695,180)	(282,340)
Other income, net	17,805	4,686	36,288	18,757
Loss before (benefit) provision for income taxes	(160,363)	(105,861)	(658,892)	(263,583)
(Benefit) provision for income taxes	(59)	(3,287)	687	(171)
Net loss	$ (160,304)	$ (102,574)	$ (659,579)	$ (263,412)
Net loss per share — basic and diluted	$ (1.70)	$ (1.50)	$ (7.50)	$ (3.86)
Weighted average common shares outstanding— basic and diluted	94,372,159	68,320,125	87,936,923	68,258,757

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CONSOLIDATED BALANCE SHEETS

(Amounts in thousands, except share amounts)

	September 30, 2024 (Unaudited)	December 31, 2023
Assets		
Current assets:		
Cash and cash equivalents	$ 84,390	$ 248,402
Marketable securities	294,426	133,417
Prepaid expenses	55,168	35,242
Income tax receivable	5,318	13,252
Other current assets	1,198	12,133
Total current assets	440,500	442,446
Property and equipment, net	18,276	17,191
Intangible assets	18,400	18,400
Goodwill	1,390	1,390
Other non-current assets	31,957	33,785
Total assets	$ 510,523	$ 513,212
Liabilities and Shareholders' Equity		
Current liabilities:		
Accounts payable	$ 19,744	$ 15,577
Accrued expenses and other current liabilities	63,520	39,846
Forward contract liability	69,030	—
Total current liabilities	152,294	55,423
Non-current operating lease liabilities	25,312	27,569
Derivative liability, non-current	12,320	—
Other non-current liabilities	4,591	2,245
Total liabilities	194,517	85,237
Shareholders' Equity:		
Preferred shares, no par value; 10,000,000 shares authorized, no shares issued and outstanding as of September 30, 2024 and December 31, 2023	—	—
Common shares, no par value; 200,000,000 shares authorized as of September 30, 2024 and December 31, 2023; 94,899,193 and 81,115,723 shares issued and outstanding as of September 30, 2024 and December 31, 2023, respectively	1,381,699	887,528
Additional paid-in capital	93,038	39,804
Accumulated deficit	(1,158,871)	(499,292)
Accumulated other comprehensive income (loss)	140	(65)
Total shareholders' equity	316,006	427,975
Total liabilities and shareholders' equity	$ 510,523	$ 513,212

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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,	
	2024	**2023**	**2024**	**2023**
Reconciliation of GAAP to Non-GAAP adjusted net loss:				
GAAP net loss	$ (160,304)	$ (102,574)	$ (659,579)	$ (263,412)
Add: non-cash share-based compensation expense	12,160	4,456	59,269	12,916
Add: (gain) loss from change in fair value of derivatives	(15,990)	—	(17,030)	—
Non-GAAP adjusted net loss	$ (164,134)	$ (98,118)	$ (617,340)	$ (250,496)
Reconciliation of GAAP to Non-GAAP adjusted net loss per share — basic and diluted:				
GAAP net loss per share — basic and diluted	$ (1.70)	$ (1.50)	$ (7.50)	$ (3.86)
Add: non-cash share-based compensation expense	0.13	0.07	0.67	0.20
Add: (gain) loss from change in fair value of derivatives	(0.17)	—	(0.19)	—
Non-GAAP adjusted net loss per share — basic and diluted	$ (1.74)	$ (1.44)	$ (7.02)	$ (3.67)

MoDEs is a trademark of Biohaven Therapeutics Ltd.
Libtayo is a registered trademark of Regeneron Pharmaceuticals, Inc.

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