Biohaven Reports Second Quarter 2024 Financial Results and Recent Business Developments

August 8, 2024

- Cash, cash equivalents, marketable securities and restricted cash totaled approximately $440 million on June 30, 2024
- Biohaven's Molecular Degrader of Extracellular Proteins (MoDE™) platform advancing multiple new targets and reported positive interim data from its lead investigational drug in an ongoing Phase 1 study of BHV-1300:
    - The Company reported dose-dependent and rapid IgG reductions in its ongoing Phase 1 trial with its lead investigational degrader BHV-1300
    - No serious adverse events (SAEs) reported with BHV-1300 to date; most adverse events (AEs) were mild, deemed unrelated to study drug and resolved spontaneously
    - Phase 1 study also completed an assessment of an optimized subcutaneous (SC) formulation of BHV-1300 that demonstrated approximately a 44% higher than expected exposure compared to the dose-equivalent intravenous formulation previously studied; this new human data further confirms feasibility of convenient self-administered SC auto-injector and the SC formulation was not associated with injection site reactions
    - Degrader platform expected to deliver 3 INDs for new MoDE programs before year-end in addition to continued data from SAD/MAD with BHV-1300
    - Biohaven's beta-1 adrenergic receptor (β1AR) autoantibody targeting MoDE, BHV-1600, granted INTERACT meeting with FDA in 2H 2024 regarding development program for dilated cardiomyopathy
- Advancing novel ion channel program targeting Kv7 activation and TRPM3 antagonism across multiple neurological, pain and neuropsychiatric disease indications:
    - 5 Phase 2/3 trials with BHV-7000 underway in epilepsy and mood disorders
    - Released positive Phase 1 data with TRPM3 antagonist BHV-2100 showing drug concentrations above EC90 target and well-tolerated profile across all doses in SAD/MAD study; advancing Phase 2 study in acute migraine and proof-of-concept (POC) study in pain in 2H 2024
- Taldefgrobep alfa, a myostatin-inhibitor, progressing on track with Phase 3 topline data in spinal muscular atrophy (SMA) and Phase 2 trial initiation in obesity expected in 2H 2024
    - Taldefgrobep alfa has demonstrated direct effects on reducing adipose tissue (including lipid storage and mitochondrial content) independent of increases in muscle mass
    - In a MAD study, conducted in healthy adults, taldefgrobep alfa (45 mg SC QW) produced significant reductions in total body fat while increasing total body lean mass
    - Preclinical data released at the 2024 American Diabetes Association conference demonstrated that taldefgrobep alfa, as a monotherapy or in combination with a GLP-1 agonist, demonstrated significant reductions in fat and total body weight. Taldefgrobep alfa-treated animals showed significant increases in lean muscle, despite co-administration with a GLP-1 receptor agonist
- Tyrosine Kinase 2/Janus Kinase 1 (TYK2/JAK1) selective inhibitor, BHV-8000, completed Phase 1 and confirmed biomarker target engagement with reductions in inflammatory markers and demonstrated central nervous system penetration with confirmed cerebrospinal fluid (CSF) target exposures in healthy subjects
    - Advancing registrational programs for Parkinson's disease and prevention of Amyloid-Related Imaging Abnormalities (ARIA) following interactions with the FDA
- Expect interim data analysis from second ongoing Phase 3 OCD trial with troriluzole in 2H 2024; topline data from first Phase 3 OCD trial expected in 1H 2025
- SCA interactions with troriluzole filing in Europe ongoing and constructive interactions in the US with the FDA
    - New real-world evidence (RWE) protocol, incorporating feedback from the FDA, assessing 3-years of treatment with troriluzole expected to deliver topline results in 2H 2024
- Biohaven antibody drug conjugate (ADC) portfolio positioned to deliver differentiated profiles and address unmet needs in oncology:
    - BHV-1510 currently dosing cancer patients in Phase1/2 study and now advancing towards combination with Libtayo® by 4Q 2024
    - Portfolio of multiple advanced nonclinical BHVN ADCs demonstrate improved plasma stability and in vitro/in vivo differentiation

NEW HAVEN, Conn., Aug. 8, 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 second quarter ended June 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, "Last quarter, we showcased an array of exciting updates across our pipeline spanning immunology, neuroscience, obesity, and oncology at our annual R&D Day. First, it has been incredible working with Yale Professor David Spiegel and my colleagues at Biohaven to drive the advancement of groundbreaking degrader science represented by BHV-1300 using our Molecular Degrader of Extracellular Protein (MoDE™) technology — we consider our initial Phase 1 data to be paradigm-shifting and we are accelerating our entire platform based upon these early results. BHV-1300 has been well-tolerated with no significant adverse effects and no clinically significant lab abnormalities or ECG changes observed to date and emerging subcutaneous data from our Phase 1 study highlights the ease of use of this technology. The platform represents a brand-new technology that has potential to treat diseases like Type 1 diabetes, autoimmune dilated cardiomyopathy, IgA nephropathy, and many others. Our differentiation continues to focus on convenient SC patient self-administration, rapid onset of action, potential for deeper IgG reduction and concurrent use with Fc containing standard of care biologics. We are excited that we remain on timelines with multiple INDs from our MoDE platform for later this year, including drug candidates that target autoantibodies against β-1AR for the potential treatment of dilated cardiomyopathy, galactose deficient IgA for IgA nephropathy and a further optimized IgG degrader for use in rare diseases."

Dr. Coric continued, "While our degrader program continues to garner significant focus, our broader pipeline is increasingly derisked with several potentially paradigm-shifting treatments for conditions including epilepsy, mood disorders, pain, immunology, rare diseases, obesity and neurodegenerative disorders. Starting with our ion channel platform; five studies with Kv7 activator BHV-7000 (*three in epilepsy and two in major depressive disorder and bipolar*) are underway with brisk patient enrollment. In addition, our TRPM3 antagonist BHV-2100 has completed its initial Phase 1 SAD/MAD study, attaining an exceptional PK/PD profile for the treatment of migraine and other pain disorders. All doses being advanced have reached the targeted EC90 for this molecule with excellent preliminary safety. We are excited about being back in the migraine space with this novel target and will initiate a Phase 2 study in migraine before year end, as well as initiate a POC study as a non-opiate pain treatment. With BHV-8000, our oral and brain penetrant TYK2/JAK1 inhibitor, we have aligned with the FDA on two novel study designs: one for the prevention of ARIA associated with amyloid lowering agents in Alzheimer's disease and another with the goal of slowing disease progression in early Parkinson's disease. The Phase 1 profile of BHV-8000 is invigorating our translational team as we have data confirming both CNS penetration and biomarker engagement of inflammatory targets in humans. Regarding our anti-myostatin taldefgrobep alfa, we shared new preclinical data demonstrating improved, durable weight reduction and lean mass preservation in combo with GLP-1 semaglutide, which was particularly noteworthy, given the muscle wasting limitations that have plagued the GLP-1 class. We also showed that taldefgrobep has direct effects on modulating fat in addition to increasing muscle mass. Our oncology platform has also emerged into the clinic with our lead Trop-2 ADC, BHV-1510, enrolling cancer patients and our efforts advancing a portfolio of several Biohaven ADCs. We are excited to get preliminary safety and efficacy data from cancer patients suffering with select advanced or metastatic epithelial tumors in the Phase 1/2 study of BHV-1510 and will be evaluating the combination of BHV-1510 with Regeneron's anti-PD1 Libtayo — combination dosing with BHV-1510 and Libtayo is anticipated to begin in 2H 2024. Finally, with our glutamate platform represented by troriluzole, topline data from an interim analysis of the second OCD trial is expected in 2H 2024 and our first OCD trial is expected to read out in 1H 2025. Separately, we continue to have constructive dialogue with the FDA regarding our SCA development program and our European Medicines Agency application for SCA3 remains under review. We now expect topline data from a new RWE protocol assessing the efficacy of troriluzole in SCA patients treated for up to 3 years— this includes new patient data that has not previously been available. I never cease to be amazed by the power of our small but mighty Biohaven R&D team that has innovated across each of these important medical areas. We are driven to fulfill a promise to patients and our investors that we will diligently evaluate and advance potentially transformative therapies to those suffering from disease."

Second Quarter 2024 and Recent Business Highlights

- ***Additional data supports the paradigm-shifting potential of the MoDE Platform for the treatment of immunological and inflammatory disorders*** – In May 2024, the Company disclosed positive new data from its ongoing Phase 1 SAD study in healthy subjects with BHV-1300, a first-in-human IgG degrader from its innovative MoDE platform. Results from the initial dose cohorts in the SAD study confirmed that BHV-1300 rapidly and selectively lowers IgG in a dose-dependent manner. Emerging Phase 1 data also showed that our novel SC formulation of BHV-1300 delivered exposures higher than the intravenous formulation, enabling the profile of a convenient patient administered auto-injector to attain targeted reduction of IgG. The Company expects to provide an update from our SAD and MAD studies of BHV-1300 in the second half of 2024 and is on schedule to file multiple new MoDE INDs this year. FDA granted INTERACT meeting request for BHV-1600, β1AR autoantibody MoDE, development program.
- ***Ion Channel Platform: Kv7 activator and TRPM3 antagonist candidates represent transformational targets in neurology and neuropsychiatry*** – The Company is enrolling participants in five ongoing pivotal clinical trials with Kv7 activator, BHV-7000, targeting focal epilepsy, generalized epilepsy, bipolar disorder and major depressive disorder; separately, Phase 2 and POC studies are planned in migraine and pain, respectively, with the TRPM3 antagonist, BHV-2100, in 2H 2024.
- ***Committed to improving muscle health with Myostatin Platform*** – In May 2024, the Company presented new preclinical data showing that administration of taldefgrobep alfa directly reduced the increased adipose fat storage caused by myostatin; the Company also presented new preclinical data from a diet-induced obesity mouse model showing treatment with taldefgrobep alfa together with a GLP-1 agonist produced greater reductions in body weight and fat mass, and a larger increase in lean muscle mass, compared to treatment with GLP-1 alone. The Company plans to initiate a

Phase 2 study in obesity in 2H2024. Separately, the Company continues to expect Phase 3 study topline results in SMA in 2H 2024.

- ***Selectively targeting the immune system to treat neurodegenerative diseases with neuroinflammation platform*** – In May 2024, the Company reported positive results from the Phase 1 SAD and MAD study with our brain-penetrant TYK2/JAK1 inhibitor, BHV-8000 in healthy subjects, including demonstration of CSF target exposures and biomarker engagement along with a well-tolerated safety profile. The Company also announced key regulatory updates, including the successful completion of two FDA meetings with favorable feedback enabling registrational programs for Parkinson's disease and for the prevention of ARIA, a novel indication.
- ***Oncology Platform: Antibody Drug Conjugate portfolio: positioned to achieve enhanced stability and improved efficacy*** – In May 2024, the Company announced that its novel Trop-2 ADC, BHV-1510, entered into the clinic for patients with advanced or metastatic epithelial tumors; the first patient was dosed in a Phase 1/2 clinical trial as monotherapy. Biohaven also entered into a clinical supply agreement with Regeneron to study the combination of BHV-1510 with Regeneron's anti-PD1 Libtayo (cemiplimab-rwlc) in the clinical study.

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:

- Topline results from a new RWE protocol of 3-year data from troriluzole treated patients in SCA in 2H 2024
- Two Phase 3 trials with troriluzole in OCD: conduct interim analysis of the second Phase 3 OCD trial in 2H 2024 and report topline data from first Phase 3 OCD trial in 1H 2025

Taldefgrobep alfa:

- Initiate Phase 2 trial with taldefgrobep in obesity in 2H 2024
- Report topline data from Phase 3 trial with taldefgrobep in SMA in 2H 2024

First-in-class TRPM3 Antagonist:

- Initiate Phase 2 trial with BHV-2100 in acute migraine in 2H 2024
- Initiate POC study with BHV-2100 for pain in 2H 2024

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 BHV-1300, with a further study update in 2H 2024
- INTERACT meeting with FDA regarding BHV-1600, β1AR autoantibody degrader, in 2H 2024

Next Generation ADC Platform:

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

Capital Position:

Cash, cash equivalents, marketable securities and restricted cash totaled approximately $440 million on June 30, 2024.

Second Quarter 2024 Financial Highlights:

Research and Development (R&D) Expenses: R&D expenses, including non-cash share-based compensation costs, were $314.8 million for the three months ended June 30, 2024, compared to $79.5 million for the three months ended June 30, 2023. The increase was primarily due to a one-time, non-cash expense of $171.9 million paid to Knopp Biosciences, LLC (Knopp) for a milestone and royalty buyback related to our BHV-7000 and broader Kv7 platform that was recognized during the three months ended June 30, 2024 (the buyback reduced our potential future milestone payments by $867.5 million, and replaced the scaled high single digit to low teens royalty payment obligations with a flat royalty payment in the mid-single digits for the Kv7 programs). The increase in R&D expenses was also due to advancing our 6 clinical platforms including 5 Phase 3 starts for BHV-7000, follow-on Kv7 assets, preclinical research programs, and increases in direct program spend for additional and advancing multiple clinical development programs. Non-cash share-based compensation expense was $7.1 million for the three months ended June 30, 2024, an increase of $4.6 million as compared to the same period in 2023. Non-cash share-based compensation expense was higher in the second 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: General and administrative expenses were $19.0 million for the three months ended June 30, 2024, compared to $14.5 million for the three months ended June 30, 2023. The increase of $4.4 million was primarily due to increased non-cash share-based compensation expense, which was $5.2 million for the three months ended June 30, 2024, an increase of $2.9 million as compared to the same period in 2023. Non-cash share-based compensation expense was higher in the second 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 a net other income of $14.2 million for the three months ended June 30, 2024, compared to a net other income of $5.8 million for the three months ended June 30, 2023. The increase of $8.3 million was primarily due to a $9.2 million gain recorded upon the settlement of our forward contract liability for share consideration issued under the amendment entered into with Knopp in May 2024, and increased investment income, partially offset by a decrease in other income recognized during the three months ended June 30, 2024, as compared to the same period in 2023 related to the Transition Services Agreement entered into with Biohaven Pharmaceutical Holding Company Ltd. (the Former Parent).

Net Loss: Biohaven reported a net loss for the three months ended June 30, 2024 of $319.8 million, or $3.64 per share, compared to $90.3 million, or $1.32 per share, for the same period in 2023. Non-GAAP adjusted net loss for the three months ended June 30, 2024 was $307.4 million, or $3.50 per share, compared to $85.7 million, or $1.25 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 (spinocerebellar ataxia); 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 June 30,		Six Months Ended June 30,	
	2024	2023	2024	2023
Operating expenses:				
Research and development	$ 314,819	$ 79,490	$ 470,791	$ 142,951
General and administrative	18,953	14,521	46,221	28,842

Total operating expenses	333,772	94,011	517,012	171,793
Loss from operations	(333,772)	(94,011)	(517,012)	(171,793)
Other income, net	14,178	5,842	18,483	14,071
Loss before provision for income taxes	(319,594)	(88,169)	(498,529)	(157,722)
Provision for income taxes	177	2,177	746	3,116
Net loss	$ (319,771)	$ (90,346)	$ (499,275)	$ (160,838)
Net loss per share — basic and diluted	$ (3.64)	$ (1.32)	$ (5.93)	$ (2.36)
Weighted average common shares outstanding— basic and diluted	87,766,069	68,248,023	84,174,099	68,227,564

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CONSOLIDATED BALANCE SHEETS

(Amounts in thousands, except share amounts)

	June 30, 2024 (Unaudited)	December 31, 2023
Assets		
Current assets:		
Cash and cash equivalents	$ 239,147	$ 248,402
Marketable securities	197,801	133,417
Prepaid expenses	59,532	35,242
Income tax receivable	7,522	13,252
Other current assets	7,266	12,133
Total current assets	511,268	442,446
Property and equipment, net	18,665	17,191
Intangible assets	18,400	18,400
Goodwill	1,390	1,390
Other non-current assets	32,918	33,785
Total assets	$ 582,641	$ 513,212
Liabilities and Shareholders' Equity		
Current liabilities:		
Accounts payable	$ 17,259	$ 15,577
Accrued expenses and other current liabilities	57,728	39,846
Forward contract and derivative liabilities	81,220	—
Total current liabilities	156,207	55,423
Non-current operating lease liabilities	26,193	27,569
Derivative liability, non-current	12,180	—
Other non-current liabilities	4,321	2,245
Total liabilities	198,901	85,237
Shareholders' Equity:		
Preferred shares, no par value; 10,000,000 shares authorized, no shares issued and outstanding as of June 30, 2024 and December 31, 2023	—	—
Common shares, no par value; 200,000,000 shares authorized as of June 30, 2024 and December 31, 2023; 92,346,332 and 81,115,723 shares issued and outstanding as of June 30, 2024 and December 31, 2023, respectively	1,298,553	887,528
Additional paid-in capital	83,832	39,804
Accumulated deficit	(998,567)	(499,292)
Accumulated other comprehensive loss	(78)	(65)
Total shareholders' equity	383,740	427,975
Total liabilities and shareholders' equity	$ 582,641	$ 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 June 30,		Six Months Ended June 30,	
	2024	**2023**	**2024**	**2023**
Reconciliation of GAAP to Non-GAAP adjusted net loss:				
GAAP net loss	$ (319,771)	$ (90,346)	$ (499,275)	$ (160,838)
Add: non-cash share-based compensation expense	12,232	4,695	47,109	8,460
Add: loss from change in fair value of derivatives	110	—	110	—
Non-GAAP adjusted net loss	$ (307,429)	$ (85,651)	$ (452,056)	$ (152,378)
Reconciliation of GAAP to Non-GAAP adjusted net loss per share — basic and diluted:				
GAAP net loss per share — basic and diluted	$ (3.64)	$ (1.32)	$ (5.93)	$ (2.36)
Add: non-cash share-based compensation expense	0.14	0.07	0.56	0.13
Add: loss from change in fair value of derivatives	—	—	—	—
Non-GAAP adjusted net loss per share — basic and diluted	$ (3.50)	$ (1.25)	$ (5.37)	$ (2.23)

MoDEs is a trademark of Biohaven Therapeutics Ltd.

Libtayo is a registered trademark of Regeneron Pharmaceuticals, Inc.

Investor Contact:

Jennifer Porcelli
Vice President, Investor Relations
jennifer.porcelli@biohavenpharma.com
+1 (201) 248-0741

Media Contact:

Mike Beyer
Sam Brown Inc.
mikebeyer@sambrown.com
+1 (312) 961-2502

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