Biohaven Reports Second Quarter 2025 Financial Results and Recent Business Developments

August 11, 2025

- Cash, cash equivalents, marketable securities and restricted cash as of June 30, 2025, totaled approximately $408.2 million
- VYGLXIA NDA for spinocerebellar ataxia (SCA) PDUFA date 4Q2025, completed clinical trial inspections by FDA without observations or findings, and filing review remains ongoing
- MoDE and TRAP degrader platforms advance in clinical development, IgG reductions of up to 87% observed with MoDE degrader BHV-1300 in Phase 1 with the potential to address IgG-mediated diseases including Graves' disease and rheumatoid arthritis; sustained and deep Gd-IgA1 reductions over 80% reported with TRAP degrader BHV-1400 highlight its potential for treating IgA Nephropathy
- Next -generation Trop2 Antibody Drug Conjugate (ADC) BHV-1510 demonstrated early clinical activity, favorable PK and differentiated safety profile in a Phase 1/2 study as a monotherapy and in combination with Regeneron's anti-PD-1 cemiplimab (Libtayo®); Tumor reduction was observed in first 6 out of 6 patients treated with BHV-1510 plus cemiplimab including confirmed partial responses
- Commenced dosing with BHV-1530, a novel FGFR3-directed ADC with potential application in urothelial cancers and other FGFR3-expressing solid tumors
- Compassionate use of opakalim (BHV-7000) in a child with intractable epilepsy due to Kv7 gene mutation (KCNQ2 Developmental and Epileptic Encephalopathy [KCNQ2-DEE]) provides early evidence of potential clinical benefit associated with Biohaven's next-generation Kv7 activator
- Enrolled first patient in pivotal Phase 2/3 study in Parkinson's disease (PD) with BHV-8000, a highly selective, brain-penetrant TYK2/JAK1 inhibitor with the potential to modulate critical inflammatory pathways that underpin the widespread immune dysregulation and neurodegeneration that drive functional decline in people with PD

NEW HAVEN, Conn., Aug. 11, 2025 /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, 2025, and provided a review of recent accomplishments and anticipated upcoming developments.

Vlad Coric, M.D., Chairman and Chief Executive Officer of Biohaven, commented, "As we eagerly await a regulatory decision on the VYGLXIA® (troriluzole) NDA for spinocerebellar ataxia, Biohaven has made important progress on multiple clinical stage assets this quarter, highlighted by the momentum we showcased at our recent R&D Day, where we unveiled advancements across our innovative therapeutic platforms." Dr. Coric added, "We are excited about the prospects of launching the first treatment for SCA if VYGLXIA is approved by the FDA and our commercial team is taking the appropriate steps to ensure an efficient launch to meet this high unmet need. We are also enthusiastic about the progress made across our Inflammation and Immunology (I&I) platform where we have observed compelling evidence of targeted protein degradation with our MoDE and TRAP degraders, BHV-1300 and BHV-1400. The body of evidence we have presented to date, combined with the safety profiles observed and convenient subcutaneous administration, continues to support our belief in our degrader platform's ultimate potential in addressing a range of immune-mediated diseases. We are also very pleased with the advancement of another key pillar of our I&I platform -- the brain-penetrant, TYK2/JAK1 inhibitor, BHV-8000, which has the potential to revolutionize the treatment of neuroinflammatory and neurodegenerative diseases. We initiated a pivotal Phase 2/3 study in Parkinson's disease, an unrelenting illness for which there is an urgent need for novel therapies to halt the progression of the disease."

Dr. Coric continued, "We also continue to take bold steps in other therapeutic areas to address important unmet medical needs for patients including in our Ion Channel and Oncology platforms. Our Kv7 platform continues to advance clinical programs toward completion in epilepsy and depression, and we are pleased to hear the promising early observations of a DEE pediatric patient successfully transitioned from ezogabine to opakalim. Finally, our Oncology platform is also generating early promising clinical data, demonstrating tumor reduction in the first 6 out of 6 patients treated with BHV-1510 plus cemiplimab. Our oncology team has also been the first to advance an FGFR3-directed ADC with potential application in urothelial cancers into clinical testing."

"Biohaven is committed to following cutting edge science to attempt to help patients across multiple areas of high unmet need. For the balance of the year, we expect to deliver continued excellence in study execution and patient enrollment across key trials in our portfolio, as well as prepare for the potential commercialization of VYGLXIA in SCA if approved. We believe the SCA data supports approval in this rare, progressive and fatal indication

for which no other treatments are available. Biohaven is well-positioned to execute on our commitment to transforming the treatment landscape for patients with serious and underserved diseases and we are excited to deliver on our promise to advance our programs for patients, caregivers, and shareholders in the balance of the year."

Second Quarter 2025 and Recent Business Highlights

- **Released new data with MoDE (*Molecular Degrader of Extracellular Proteins*) program:** In May 2025, the Company released new positive data from the Phase 1 study of BHV-1300, a MoDE initially being developed for the treatment of common immune-mediated diseases, such as Graves' disease and rheumatoid arthritis. In the Phase 1 multiple-dose study, subcutaneous administered BHV-1300 achieved IgG reductions up to 87%. Median maximum reductions of 83% were achieved within 18 days. The range of IgG lowering enabled by different dose levels of BHV-1300 potentially offers tunability and flexibility in dosing paradigm, with higher doses planned for management of acute conditions, and lower, less frequent dosing planned for the management of chronic disease.
- **Released new data with TRAP (*Targeted Removal of Aberrant Protein*) degrader program:** In May 2025, the Company announced further data from the Phase 1 study of BHV-1400, a TRAP degrader initially being developed to target Gd-IgA1, the aberrant immunoglobulin that drives IgA Nephropathy. In the Phase 1 study, a single dose of BHV-1400 was subcutaneously administered at a dose of 500 mg and achieved rapid, deep and sustained reductions in Gd-IgA1 of up to 81%, with a median reduction of 66%. Reductions occurred within hours of each dose, were progressive, and were sustained for weeks after a single dose administration. Effects were selective, with no significant reductions observed in other immunoglobulins (IgA, IgG, IgE, or IgM).
- **Demonstrated early clinical activity and favorable PK profile with BHV-1510:** In May 2025, the Company announced early clinical results from a Phase 1 study of BHV-1510, a next-generation Trop2-directed ADC incorporating the proprietary TopoIx payload. The data demonstrated early signs of clinical activity and a differentiated, manageable safety profile, both as monotherapy and in combination with Regeneron's anti-PD-1, cemiplimab. Partial responses were observed across multiple tumor types with BHV-1510 monotherapy, accompanied by low rates of payload-related toxicities. The most common adverse event was stomatitis, an anticipated and manageable class effect associated with Trop2-targeted therapies. Notably, tumor reduction was seen in all six of the first patients treated with the BHV-1510 and cemiplimab combination, including confirmed partial responses. The combination regimen was well tolerated, with no dose-limiting toxicities or cases of interstitial lung disease reported in these initial cohorts. These encouraging early clinical data, along with the favorable pharmacokinetic profile and proprietary stable linker technology, support continued investigation of BHV-1510 as monotherapy and in combination with cemiplimab in difficult-to-treat tumor types, including potential evaluation in earlier lines of therapy for patients with advanced or metastatic disease.
- **First patient dosed with Biohaven's TopoIx ADC, BHV-1530:** In May 2025, the Company commenced dosing with BHV-1530, a potential first-in-class fibroblast growth factor receptor 3 (FGFR3)-directed ADC which utilizes the proprietary TopoIx payload. BHV-1530 has potential in cancer indications driven by FGFR3 alterations and/or upregulated FGFR3 protein expression, including urothelial cancers and other solid tumors.
- **Phase 2/3 PD trial initiated:** In May 2025, the Company commenced enrollment in a global, pivotal, Phase 2/3 study of the first-in-clinic, orally-administered, brain-penetrant, and highly selective TYK2/JAK1 inhibitor, BHV-8000, for the treatment of early Parkinson's disease (PD).
- **Compassionate use of opakalim (BHV-7000) in a child with intractable epilepsy due to Kv7 gene mutation (KCNQ2 Developmental and Epileptic Encephalopathy [KCNQ2-DEE]) provides early evidence of potential clinical benefit.** A child with KCNQ2-DEE and a history of intractable epilepsy who was previously maintained on ezogabine, as well as other antiepileptics, was successfully transitioned to treatment with opakalim, Biohaven's next-generation Kv7 activator. Opakalim was administered after receiving a compassionate use request, under a single patient IND approved by the FDA, as the child was being withdrawn from ezogabine treatment. The child had multiple unsuccessful attempts in the past to taper ezogabine, leading to severe seizure exacerbations requiring admission to the hospital intensive care unit. Dosing of opakalim in this pediatric patient was selected to achieve comparable exposures as the 75mg dose being investigated in ongoing Phase 2/3 clinical trials. Following the transition, the patient demonstrated signs of therapeutic benefit as assessed by initial seizure control and a favorable side effect profile. Although generalizability of these observations is limited, given it represents a single case report of treating a KCNQ2-DEE patient and a short initial follow-up period after transition from ezogabine, the early clinical experience after initiation of opakalim, a selective Kv7 activator, is promising for its observed antiseizure effects and favorable tolerability.
- **Phase 3 trial in OCD with troriluzole was completed with no efficacy signal detected.** The OCD development program is being ended to allow resources to be applied to other development programs. Study results will be presented at an upcoming academic meeting.

Expected Upcoming Milestones:

We believe Biohaven is well positioned to achieve significant milestones in 2025 and 2026 across numerous programs:

MoDE Platform

- IgG MoDE Degraders (1300/1310): Initiated Phase 1b study in Graves' disease in 2H 2025, with potentially registrational

study expected to initiate in 2H 2025.
- Phase 1 studies in healthy volunteers with BHV-1400 and BHV-1600 concluding, with BHV-1400 Phase 1 studies expanding to include patients with IgA nephropathy. BHV-1400 potentially registrational study expected to initiate in 2026.
- Four additional degrader molecules advancing, including: IgG4 degrader, PLA2R autoantibody degrader, pro-insulin autoantibody degrader, and TSH receptor autoantibody degrader.

Kv7 Activator (BHV-7000):

- Pivotal major depressive disorder topline results expected in 2H 2025.
- Focal epilepsy study pivotal topline results expected in 1H 2026.

Glutamate Modulator (VYGLXIA):

- Priority Review of SCA NDA ongoing, with PDUFA expected in 4Q 2025. Preparing for potential commercial launch in all-genotype SCA if approved by FDA.

Myostatin (Taldefgrobep alfa):

- Continue ongoing Health Authority interactions to discuss Spinal Muscular Atrophy ("SMA") registrational path in the U.S. and Europe.
- Expect to initiate Phase 2 study in obesity in 2H 2025.

TYK2/JAK1 Inhibitor (BHV-8000):

- Continue advancing enrollment in Phase 2/3 study in Parkinson's disease.
- Advance Alzheimer's disease, multiple sclerosis ("MS") and amyloid-related imaging abnormalities ("ARIA") programs.

Next Generation ADC Platform:

- Continue advancing Phase 1/2 study with BHV-1510 as monotherapy and combination therapy with cemiplimab in epithelial tumors in 2025.
- Continue advancing Phase 1 study with BHV-1530, FGFR3-directed ADC utilizing proprietary Topolx payload with potential applications in urothelial cancers and other solid tumors.
- Advance additional preclinical ADCs, including Merus and GeneQuantum collaborations (undisclosed targets) in 2025.

Capital Position:

Cash, cash equivalents, marketable securities and restricted cash as of June 30, 2025 totaled approximately $408.2 million.

Second Quarter 2024 Financial Highlights:

Research and Development (R&D) Expenses: R&D expenses, including non-cash share-based compensation costs, were $184.4 million for the three months ended June 30, 2025, compared to $314.8 million for the three months ended June 30, 2024. The decrease of $130.5 million was primarily due to a one-time non-cash expense during the three months ended June 30, 2024, paid to Knopp for a milestone and royalty buyback related to the BHV-7000 and broader Kv7 platform. The decrease was partially offset by increased direct program costs for advancing clinical trials and preclinical research programs in 2025, including one-time developmental milestone payments of $15.0 million and $10.0 million for our BHV-8000 and BHV-1530 programs, respectively, as well as increased non-cash share-based compensation expense. Non-cash share-based compensation expense was $13.1 million for the three months ended June 30, 2025, an increase of $6.0 million as compared to the same period in 2024. Non-cash share-based compensation expense was higher in 2025 primarily due to our annual equity incentive awards granted in the first quarter of 2025.

General and Administrative (G&A) Expenses: G&A expenses, including non-cash share-based compensation costs, were $27.3 million for the three months ended June 30, 2025, compared to $19.0 million for the three months ended June 30, 2024. The increase of $8.4 million was primarily due to increased non-cash share-based compensation expense and increased expenses related to fees incurred in connection with the Note Purchase Agreement with Beetlejuice SA LLC, an affiliate of Oberland Capital Management LLC, entered into during the second quarter of 2025 (the Note Purchase Agreement) and other legal costs. Non-cash share-based compensation expense was $7.7 million for the three months ended June 30, 2025, an increase of $2.5 million as compared to the same period in 2024. Non-cash share-based compensation expense was higher in 2025 primarily due to our annual equity incentive awards granted in the first quarter of 2025.

Other Income, Net: Other income, net was $13.8 million for the three months ended June 30, 2025, compared to other income, net of $14.2 million for the three months ended June 30, 2024. The decrease of $0.4 million was primarily due to decreased investment income and an increase in non-cash losses related to changes in fair value of our notes payable liability under the Note Purchase Agreement during the second quarter of 2025, partially offset by an increase in gains recorded for the non-cash changes in the fair value of our forward contract and derivative liability recorded in connection with the amendment to our Membership Interest Purchase Agreement with Knopp Biosciences LLC in May 2024 (the Knopp Amendment).

Net Loss: Biohaven reported a net loss for the three months ended June 30, 2025 of $198.1 million, or $1.94 per share, compared to $319.8 million, or $3.64 per share, for the same period in 2024. Non-GAAP adjusted net loss for the three months ended June 30, 2025 was $166.4 million, or $1.63 per share, compared to $308.6 million, or $3.52 per share, for the same period in 2024. 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; MoDE™ and TRAP™ extracellular protein degradation for immunological diseases; TRPM3 antagonism for neuropathic pain; TYK2/JAK1 inhibition for neuroinflammatory disorders; glutamate modulation for 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, including statements regarding the expected timing and amounts of funding under the NPA. 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 and the expected timing thereof; the potential for Biohaven's product candidates to be successful 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.

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

	Three Months Ended June 30,		Six Months Ended June 30,	
	2025	2024	2025	2024
Operating expenses:				
Research and development	$ 184,367	$ 314,819	$ 371,951	$ 470,791
General and administrative	27,334	18,953	61,311	46,221
Total operating expenses	211,701	333,772	433,262	517,012
Loss from operations	(211,701)	(333,772)	(433,262)	(517,012)
Other income, net	13,815	14,178	14,308	18,483
Loss before provision for income taxes	(197,886)	(319,594)	(418,954)	(498,529)
Provision for income taxes	261	177	870	746
Net loss	$ (198,147)	$ (319,771)	$ (419,824)	$ (499,275)
Net loss per share — basic and diluted	$ (1.94)	$ (3.64)	$ (4.11)	$ (5.93)
Weighted average common shares outstanding— basic and diluted	102,372,820	87,766,069	102,159,294	84,174,099

BIOHAVEN LTD.
CONSOLIDATED BALANCE SHEETS
(Amounts in thousands, except share amounts)

	June 30, 2025 (Unaudited)	December 31, 2024
Assets		

Current assets:		
Cash and cash equivalents	$ 165,797	$ 99,134
Marketable securities	239,183	386,857
Prepaid expenses	61,489	49,376
Other current assets	8,358	3,105
Total current assets	474,827	538,472
Property and equipment, net	18,593	17,320
Intangible assets	18,400	18,400
Goodwill	1,390	1,390
Other non-current assets	37,205	39,525
Total assets	$ 550,415	$ 615,107
Liabilities and Shareholders' Equity		
Current liabilities:		
Accounts payable	$ 19,628	$ 18,029
Accrued expenses and other current liabilities	78,726	51,487
Forward contract and derivative liability	25,970	84,710
Total current liabilities	124,324	154,226
Non-current operating lease liability	29,797	32,782
Notes payable	257,070	—
Other non-current liabilities	4,637	4,663
Total liabilities	415,828	191,671
Shareholders' Equity:		
Preferred shares, no par value; 10,000,000 shares authorized, no shares issued and outstanding as of June 30, 2025 and December 31, 2024	—	—
Common shares, no par value; 200,000,000 shares authorized as of June 30, 2025 and December 31, 2024; 105,782,447 and 101,221,989 shares issued and outstanding as of June 30, 2025 and December 31, 2024, respectively	1,743,109	1,656,702
Additional paid-in capital	157,019	112,369
Accumulated deficit	(1,765,538)	(1,345,714)
Accumulated other comprehensive (loss) income	(3)	79
Total shareholders' equity	134,587	423,436
Total liabilities and shareholders' equity	$ 550,415	$ 615,107

BIOHAVEN LTD.
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,	
	2025	**2024**	**2025**	**2024**
Reconciliation of GAAP to Non-GAAP adjusted net loss:				
GAAP net loss	$ (198,147)	$ (319,771)	$ (419,824)	$ (499,275)
Add: non-cash share-based compensation expense	20,812	12,232	73,874	47,109
Add: loss from change in fair value of derivatives	10,970	(1,040)	12,760	(1,040)
Non-GAAP adjusted net loss	$ (166,365)	$ (308,579)	$ (333,190)	$ (453,206)
Reconciliation of GAAP to Non-GAAP adjusted net loss per share — basic and diluted:				
GAAP net loss per share — basic and diluted	$ (1.94)	$ (3.64)	$ (4.11)	$ (5.93)
Add: non-cash share-based compensation expense	0.20	0.14	0.72	0.56
Add: loss from change in fair value of derivatives	0.11	(0.01)	0.12	(0.01)
Non-GAAP adjusted net loss per share — basic and diluted	$ (1.63)	$ (3.52)	$ (3.26)	$ (5.38)

VYGLXIA is a registered trademark, and MoDE and TRAP are trademarks, of Biohaven Therapeutics Ltd.

Libtayo is a registered trademark of Regeneron Pharmaceuticals, Inc.

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