



Wave Life Sciences Reports Second Quarter 2025 Financial Results and Provides Business Update

July 30, 2025

WVE-006, the leading GalNAc-RNA editing oligonucleotide designed to correct AAT protein and address both lung and liver manifestations of AATD, rapidly advancing in RestorAATion-2 study of AATD patients; multi-dosing is complete in first cohort (200 mg) and single dosing is complete in second cohort (400 mg)

RestorAATion-2 clinical data from complete 200 mg single and multidose dose cohorts remain on track for 3Q 2025; data from complete 400 mg single dose cohort remain on track for fall 2025

WVE-007, an INHBE GalNAc-siRNA designed to induce fat loss with muscle preservation, dosing is complete in expanded Cohort 2 (240 mg; from 8 to 32 individuals) of INLIGHT trial at dose predicted to be therapeutically active based on preclinical weight loss data

Expansion of INLIGHT Cohort 2 was triggered by favorable safety and tolerability as well as robust Activin E reduction in Cohort 1 (75 mg; n=8); Cohort 1 and 2 data expected in 4Q 2025 and dosing underway in Cohort 3 (400 mg) with data expected in 1Q 2026

Cash and cash equivalents of \$208.5 million as of June 30, 2025, with runway expected into 2027

Investor conference call and webcast at 8:30 a.m. ET today

CAMBRIDGE, Mass., July 30, 2025 (GLOBE NEWSWIRE) -- Wave Life Sciences Ltd. (Nasdaq: WVE), a clinical-stage biotechnology company focused on unlocking the broad potential of RNA medicines to transform human health, today announced financial results for the second quarter ended June 30, 2025, and provided a business update.

"We have rapidly advanced our RestorAATion-2 study following positive proof-of mechanism data last year where we observed mean total AAT protein that met the level that has been the basis for regulatory approval for AAT augmentation therapies following a single, lowest planned dose of WVE-006, our GalNAc-RNA editing candidate for AATD," said Paul Bolno, MD, MBA, President and Chief Executive Officer at Wave Life Sciences. "We remain on track to share two comprehensive data sets from our RestorAATion-2 trial this year, beginning with multidose data in the third quarter, which will inform the therapeutic potential of WVE-006, and our pipeline of wholly-owned GalNAc-RNA editing programs."

Dr. Bolno continued, "We are also advancing our INLIGHT trial of WVE-007, our INHBE GalNAc-siRNA that uses our propriety chemistry and best-in-class design, in individuals living with overweight and obesity. Since our last update, we have expanded Cohort 2 based on the favorable safety and tolerability data and robust target engagement we observed in Cohort 1, our lowest single dose cohort, and dosed 24 additional patients in Cohort 2. This achievement confirms the successful clinical translation of our siRNA platform and preclinical modeling. It also strengthens our conviction in upcoming data from Cohort 2, which tests a dose that is projected to be therapeutically active based on preclinical weight loss data. Additionally, WVE-N531, for DMD, and WVE-003, for HD, remain on track, both of which have demonstrated potential best-in-class therapeutic profiles. Our consistent execution in the clinic has positioned us to deliver multiple robust data sets in the second half of 2025 that carry the potential to further extend our leadership in RNA medicines."

Recent Business Highlights and Expected Milestones

AATD (Alpha-1 antitrypsin deficiency)

- **WVE-006** is a GalNAc-conjugated, subcutaneously delivered, A-to-I RNA editing oligonucleotide (AIMer) that is uniquely designed to address alpha-1 antitrypsin deficiency (AATD)-related lung disease, liver disease, or both.
- **RestorAATion clinical program:** RestorAATion-1 (healthy volunteers) completed in 2024 and RestorAATion-2 (Phase 1b/2a open-label study with both single and multiple ascending dose portions) is ongoing and evaluating the safety, tolerability, pharmacodynamics and pharmacokinetics of WVE-006 in individuals with AATD who have the homozygous Pi*ZZ mutation.
 - Positive proof-of-mechanism data was announced in October 2024 from a single, lowest dose of WVE-006 from the first two patients in the ongoing RestorAATion-2 clinical study, representing the first-ever clinical demonstration of RNA editing in humans. Circulating wild-type M-AAT protein in plasma reached a mean of 6.9 micromolar, representing more than 60% of total AAT. Mean total AAT protein increased to 10.8 micromolar, meeting the level that has been the basis for regulatory approval for AAT augmentation therapies. In addition, increases in AAT from baseline were observed as early as Day 3 and as late as Day 57.
 - Multi-dosing is complete in the first cohort of RestorAATion-2, where patients received 200 mg subcutaneous doses every two weeks and single dosing is complete in the second single dose cohort at 400 mg.
- **Expected milestones:** Wave expects to share data from the complete 200 mg single and multidose cohorts of RestorAATion-2 in the third quarter of 2025, and data from the complete 400 mg single dose cohort in the fall of 2025.

Obesity

- **WVE-007** is a GalNAc-conjugated small interfering RNA (GalNAc-siRNA) designed to silence INHBE mRNA, an obesity target with strong evidence from human genetics. WVE-007 is Wave's first siRNA candidate to enter clinical development and uses Wave's best-in-class proprietary oligonucleotide chemistry. Published [preclinical data](#) on Wave's siRNA designs demonstrate unprecedented Ago2 loading following a single dose of Wave's siRNA, leading to improved potency and durability *in vivo* versus comparator siRNA formats.
- **INLIGHT** is an ongoing, first-in-human clinical trial (3:1 active: placebo) evaluating WVE-007 in adults living with overweight or obesity and assesses safety, tolerability, pharmacokinetics, biomarkers for target engagement, body weight and composition, and metabolic health.
 - In the second quarter of 2025, Cohort 2 of INLIGHT, which is evaluating single 240 mg doses of WVE-007, was expanded from eight to 32 individuals. This expansion was triggered by favorable safety and tolerability, as well as robust Activin E reduction observed in Cohort 1 (75 mg; n=8), the lowest single dose cohort. The 240 mg dose level is predicted to be therapeutically active based on Wave's preclinical diet-induced obesity (DIO) mice data, where a single dose of INHBE GalNAc-siRNA led to potent and durable reductions of both INHBE mRNA and Activin E protein and drove weight loss.
 - Today, Wave announced dosing is complete for these additional participants in the expanded Cohort 2 and data from both Cohort 1

- and Cohort 2 are expected in the fourth quarter of 2025.
- o Dosing is also underway in Cohort 3 (400 mg) with data anticipated in the first quarter of 2026.
- In [June 2025](#), at the American Diabetes Association's 85th Annual Scientific Sessions, Wave presented its preclinical DIO data that support WVE-007's potential to reduce fat, preserve muscle and induce healthy weight loss with dosing once or twice a year. Key highlights include:
 - o A single dose of INHBE GalNAc-siRNA led to potent and durable reductions of both INHBE mRNA and Activin E protein, which drove weight loss on par with semaglutide with preserved muscle mass.
 - o A single dose of INHBE GalNAc-siRNA led to shrinkage of adipocytes and lower inflammatory state of visceral adipose tissues in DIO mice, with strong suppression of pro-inflammatory M1 macrophages in visceral fat of DIO mice, highlighting potential mechanistic insights into the risk reduction for type 2 diabetes and coronary artery disease suggested by human genetics.
- **Expected milestones:** Wave expects to deliver data from the expanded Cohort 2 (240 mg) as well as data from Cohort 1 (75mg) of INLIGHT in the fourth quarter of 2025, including safety, tolerability and measurements reflective of healthy weight loss. Data from Cohort 3 (400 mg) of INLIGHT are anticipated in the first quarter of 2026.

Emerging wholly owned siRNA and RNA editing pipeline

- Wave is advancing new targets across multiple disease areas to expand its pipeline of wholly owned programs in both rare and common diseases. Wave's pipeline of preclinical candidates utilizes Wave's proprietary chemistry to achieve best-in-class siRNA silencing and RNA editing in a variety of hepatic and extrahepatic tissues. Within RNA editing, Wave has demonstrated the ability to correct single variants to restore wild-type protein function and to increase the stability of the mRNA transcript to upregulate protein levels.
- Wave plans to host a Research Day in the fall of 2025, where the company expects to share updates from its clinical and preclinical pipeline of RNA medicines.

DMD (Duchenne muscular dystrophy)

- **WVE-N531** is an exon skipping oligonucleotide being developed as a disease modifying treatment for boys with Duchenne muscular dystrophy amenable to exon 53 skipping. WVE-N531 was designed using Wave's best-in-class oligonucleotide chemistry modifications, including PN backbone chemistry. WVE-N531 has received Orphan Drug Designation and Rare Pediatric Disease Designation from the U.S. Food & Drug Administration.
- In a positive Phase 2, open label clinical trial, boys receiving WVE-N531 for 48 weeks had a statistically significant and clinically meaningful improvement in Time-to-Rise vs. natural history, the first-ever demonstration of substantial improvements in muscle health with exon skipping. Additionally, WVE-N531 was safe and well-tolerated with no Serious Adverse Events.
- **Expected milestones:** Wave plans to file a New Drug Application (NDA) in 2026 to support accelerated approval of WVE-N531 with monthly dosing. Wave expects to submit clinical trial applications (CTAs) for additional exon skipping programs in 2026.

HD (Huntington's disease)

- **WVE-003** is a first-in-class, allele-selective oligonucleotide for the treatment of Huntington's disease (HD). In the SELECT-HD clinical trial, data demonstrated the first-ever allele-selective reduction in CSF mHTT protein and preservation of healthy, wtHTT with multiple doses of WVE-003, as well as a statistically significant correlation between mHTT reduction and slowing of caudate atrophy. By reducing mHTT at the mRNA and protein level, WVE-003 addresses underlying drivers of neurodegeneration. In addition, by sparing wtHTT protein, which is critical to the health of the central nervous system, WVE-003 is uniquely positioned to address individuals living with HD who are presymptomatic as well as those who are symptomatic.
- Wave has received supportive initial feedback from FDA, who recognize the severity of HD and are receptive to and engaged with Wave regarding a potential pathway to accelerated approval. Preparation is ongoing for a potentially registrational, global Phase 2/3 study of WVE-003 in adults with SNP3 and HD using caudate atrophy as a primary endpoint.
- **Expected milestones:** Wave expects to submit an Investigational New Drug (IND) application for a potentially registrational Phase 2/3 study of WVE-003 in HD in the second half of 2025.

Financial Highlights

- Cash and cash equivalents were \$208.5 million as of June 30, 2025, compared to \$302.1 million as of December 31, 2024. Wave expects that its current cash and cash equivalents will be sufficient to fund operations into 2027. Potential future milestones and other payments to Wave under its GSK collaboration are not included in its cash runway.
- Revenue recognized was \$8.7 million for the second quarter of 2025 as compared to \$19.7 million in the prior year quarter.
- Research and development expenses were \$43.5 million in the second quarter of 2025 as compared to \$40.4 million in the same period in 2024.
- General and administrative expenses were \$18.0 million in the second quarter 2025 as compared to \$14.3 million in the same period in 2024.
- Net loss was \$50.5 million for the second quarter of 2025 as compared to \$32.9 million in the prior year quarter.

Corporate Highlights

- In May 2025, Wave announced the appointment of Christopher Wright, MD, PhD, as Chief Medical Officer leading global development, including medical, clinical, and regulatory functions. Most recently, Dr. Wright was Chief Medical Officer at Ring Therapeutics. Previously, Dr. Wright held leadership roles at AavantiBio (acquired by Solid Biosciences), CycLERION Therapeutics, Ironwood Pharmaceuticals, Inc, Axcella Health Inc., and Vertex Pharmaceuticals, where he co-wrote the first ever approved application (Kalydeco®/ivacaftor) for Breakthrough Status and oversaw the submissions teams that ultimately led to successful FDA and EMA approvals. Dr. Wright spent 20 years as a practicing physician at Brigham and Women's Hospital.

Investor Conference Call and Webcast

Wave will host an investor conference call today at 8:30 a.m. ET to review the second quarter 2025 financial results and pipeline updates. A webcast of the conference call can be accessed by visiting "Investor Events" on the investor relations section of the Wave Life Sciences website: <https://ir.wavelifesciences.com/events-publications/events>. Analysts planning to participate during the Q&A portion of the live call can join the conference call at the audio-conferencing link [here](#). Following the live event, an archived version of the webcast will be available on the Wave Life Sciences website.

About Wave Life Sciences

Wave Life Sciences (Nasdaq: WVE) is a biotechnology company focused on unlocking the broad potential of RNA medicines to transform human health. Wave's RNA medicines platform, PRISM®, combines multiple modalities, chemistry innovation and deep insights in human genetics to deliver scientific breakthroughs that treat both rare and common disorders. Its toolkit of RNA-targeting modalities includes editing, splicing, RNA interference and antisense silencing, providing Wave with unmatched capabilities for designing and sustainably delivering candidates that optimally address disease biology. Wave's diversified pipeline includes clinical programs in alpha-1 antitrypsin deficiency, obesity, Duchenne muscular dystrophy, and

Huntington's disease, as well as several preclinical programs utilizing the company's broad RNA therapeutics toolkit. Driven by the calling to "Reimagine Possible", Wave is leading the charge toward a world in which human potential is no longer hindered by the burden of disease. Wave is headquartered in Cambridge, MA. For more information on Wave's science, pipeline and people, please visit www.wavelifesciences.com and follow Wave on [X](#) (formerly Twitter) and [LinkedIn](#).

Forward-Looking Statements

This press release contains forward-looking statements concerning our goals, beliefs, expectations, strategies, objectives and plans, and other statements that are not necessarily based on historical facts, including statements regarding the following, among others: the anticipated initiation, site activation, patient recruitment, patient enrollment, dosing, generation and reporting of data and completion of our clinical trials, including interactions with regulators and any potential registration based on these data, and the timing and announcement of such events; the protocol, design, endpoints, dose levels and dosing frequency for our investigational therapeutics in clinical trials; the future performance and results of our programs in clinical trials, including the anticipated therapeutic benefits of such programs; our expectations with respect to how our clinical data successes to date may predict success for our future therapeutic candidates and data readouts and may further validate our platform; preclinical activities and programs and their potential to transition into clinical-stage programs, and the timing and announcement of data related to such preclinical activities; the potential of our preclinical data to predict the behavior of our compounds in humans; regulatory submissions and timing for regulatory feedback; the submission of marketing approval applications to regulators, approval thereof, and the potential commercialization of our late-stage programs; the progress and potential benefits of collaborations and strategic partnerships; the potential achievement of milestones under any collaborations; our identification of future product candidates and their therapeutic potential; the status and progress of our programs and the anticipated benefits of our therapeutic candidates and pipeline compared to our competitors; the potential unmet medical needs and addressable patient population estimates related to our therapeutic candidates; our ability to design compounds using the most appropriate of our multiple modalities and the anticipated benefits of that approach; the breadth and versatility of our drug discovery and development platform; the expected benefits of our stereopure oligonucleotides compared with stereorandom oligonucleotides; the benefits of RNA medicines generally; the potential benefits of our RNA editing capability, including our AIMers, compared to others; the potential for certain of our programs to be best-in-class or first-in-class, or to change the existing treatment paradigm or show substantial benefits over existing standards of care; anticipated benefits of our proprietary manufacturing processes and our internal manufacturing capabilities; the strength of our intellectual property and the data that support our IP; the anticipated duration of our cash runway and our ability to fund future operations; our intended uses of capital; and our expectations regarding the impact of any potential global macro events on our business. Actual results may differ materially from those indicated by these forward-looking statements as a result of various important factors, including the following: our ability to finance our drug discovery and development efforts and to raise additional capital when needed; the ability of our preclinical programs to produce data sufficient to support our clinical trial applications and the timing thereof; the clinical results of our programs and the timing thereof, which may not support further development of our product candidates; actions of regulatory authorities and their receptiveness to our trial designs and accelerated approval pathways, which may affect the initiation, timing and progress of clinical trials; our effectiveness in managing interactions with regulatory authorities; the effectiveness of our drug discovery and development platform; the effectiveness of our RNA editing capability and our AIMers; our ability to demonstrate the therapeutic benefits of our candidates in clinical trials, including our ability to develop candidates across multiple therapeutic modalities; our dependence on third parties, including contract research organizations, contract manufacturing organizations, collaborators and partners; our ability to manufacture or contract with third parties to manufacture drug material to support our programs and growth; our ability to obtain, maintain and protect our intellectual property; our ability to enforce our patents against infringers and defend our patent portfolio against challenges from third parties; competition from others developing therapies for the indications we are pursuing; our ability to maintain the company infrastructure and personnel needed to achieve our goals; any potential macro economic events, including changes in economic policies; and the information under the caption "Risk Factors" contained in our most recent Annual Report on Form 10-K filed with the Securities and Exchange Commission (SEC) and in other filings we make with the SEC from time to time. We undertake no obligation to update the information contained in this press release to reflect subsequently occurring events or circumstances.

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WAVE LIFE SCIENCES LTD.
UNAUDITED CONSOLIDATED BALANCE SHEETS
(In thousands, except share amounts)

	<u>June 30, 2025</u>	<u>December 31, 2024</u>
Assets		
Current assets:		
Cash and cash equivalents	\$ 208,481	\$ 302,078
Accounts receivable	1,617	1,422
Prepaid expenses	6,700	9,544
Other current assets	<u>6,758</u>	<u>7,350</u>
Total current assets	<u>223,556</u>	<u>320,394</u>
Long-term assets:		
Property and equipment, net of accumulated depreciation of \$47,918 and \$46,329 as of June 30, 2025 and December 31, 2024, respectively	9,037	10,128
Operating lease right-of-use assets	15,250	17,870
Restricted cash	3,784	3,760

Other assets	728	55
Total long-term assets	<u>28,799</u>	<u>31,813</u>
Total assets	<u>\$ 252,355</u>	<u>\$ 352,207</u>
Liabilities, Series A preferred shares, and shareholders' equity		
Current liabilities:		
Accounts payable	\$ 14,664	\$ 16,262
Accrued expenses and other current liabilities	13,026	21,081
Current portion of deferred revenue	51,582	65,972
Current portion of operating lease liability	<u>8,136</u>	<u>7,638</u>
Total current liabilities	<u>87,408</u>	<u>110,953</u>
Long-term liabilities:		
Deferred revenue, net of current portion	4,232	6,099
Operating lease liability, net of current portion	<u>13,575</u>	<u>17,766</u>
Total long-term liabilities	<u>17,807</u>	<u>23,865</u>
Total liabilities	<u>\$ 105,215</u>	<u>\$ 134,818</u>
Series A preferred shares, no par value; 3,901,348 shares issued and outstanding at June 30, 2025 and December 31, 2024	<u>\$ 7,874</u>	<u>\$ 7,874</u>
Shareholders' equity:		
Ordinary shares, no par value; 155,673,292 and 153,037,286 shares issued and outstanding at June 30, 2025 and December 31, 2024, respectively	\$ 1,191,029	\$ 1,175,181
Additional paid-in capital	167,603	156,454
Accumulated other comprehensive loss	(161)	(262)
Accumulated deficit	<u>(1,219,205)</u>	<u>(1,121,858)</u>
Total shareholders' equity	<u>\$ 139,266</u>	<u>\$ 209,515</u>
Total liabilities, Series A preferred shares, and shareholders' equity	<u>\$ 252,355</u>	<u>\$ 352,207</u>

WAVE LIFE SCIENCES LTD.
UNAUDITED CONSOLIDATED STATEMENTS OF OPERATIONS AND COMPREHENSIVE LOSS

(In thousands, except share and per share amounts)

	<u>Three Months Ended June 30,</u>		<u>Six Months Ended June 30,</u>	
	<u>2025</u>	<u>2024</u>	<u>2025</u>	<u>2024</u>
Revenue	\$ 8,699	\$ 19,692	\$ 17,874	\$ 32,230
Operating expenses:				
Research and development	43,469	40,393	84,091	73,840
General and administrative	17,989	14,296	36,346	27,845
Total operating expenses	<u>61,458</u>	<u>54,689</u>	<u>120,437</u>	<u>101,685</u>
Loss from operations	<u>(52,759)</u>	<u>(34,997)</u>	<u>(102,563)</u>	<u>(69,455)</u>
Other income, net:				
Interest income	2,372	2,092	5,247	4,627
Other income (expense), net	(82)	(18)	(31)	347
Total other income, net	<u>2,290</u>	<u>2,074</u>	<u>5,216</u>	<u>4,974</u>
Loss before income taxes	(50,469)	(32,923)	(97,347)	(64,481)
Income tax benefit (provision)	—	—	—	—
Net loss	<u>\$ (50,469)</u>	<u>\$ (32,923)</u>	<u>\$ (97,347)</u>	<u>\$ (64,481)</u>
Net loss per share attributable to ordinary shareholders—basic and diluted	<u>\$ (0.31)</u>	<u>\$ (0.25)</u>	<u>\$ (0.60)</u>	<u>\$ (0.50)</u>
Weighted-average ordinary shares used in computing net loss per share attributable to ordinary shareholders—basic and diluted	<u>163,987,640</u>	<u>129,527,003</u>	<u>163,266,106</u>	<u>129,399,340</u>
Other comprehensive loss:				
Net loss	\$ (50,469)	\$ (32,923)	\$ (97,347)	\$ (64,481)
Foreign currency translation	43	(81)	101	(155)
Comprehensive loss	<u>\$ (50,426)</u>	<u>\$ (33,004)</u>	<u>\$ (97,246)</u>	<u>\$ (64,636)</u>

