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

November 10, 2025

WVE-007, an INHBE GalNAc-siRNA for obesity designed to drive fat loss while preserving muscle mass, achieved dose-dependent, mean reductions of Activin E of up to 85% in INLIGHT clinical trial, exceeding levels that led to weight loss and prevention of rebound weight gain following cessation of GLP-1 in preclinical models

Activin E reduction in lowest single dose cohort of INLIGHT was sustained through six months, supporting once or twice a year dosing

Achieved key AATD treatment goals to recapitulate the MZ phenotype with WVE-006, GalNAc-RNA editing oligonucleotide, in RestorAATion-2 trial: AAT protein exceeded 20 µM during an acute phase response, basal AAT levels reached 13 µM, wild-type M-AAT protein reached 64% of serum AAT, Z-AAT was reduced by 60%

WVE-N531 in DMD and WVE-003 in HD remain on track

Cash and cash equivalents of $196.2 million as of September 30, 2025; subsequent to quarter-end, additional $72.1 million in ATM proceeds and committed GSK milestones extend expected cash runway into 2Q 2027

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

CAMBRIDGE, Mass., Nov. 10, 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 third quarter ended September 30, 2025, and provided a business update.

“In the third quarter, we achieved key clinical objectives with WVE-007 for obesity and WVE-006 for alpha-1 antitrypsin deficiency, which validate the impact of our proprietary chemistry and further solidify our growing leadership in RNAi and RNA editing,” said Paul Bolno, MD, MBA, President and Chief Executive Officer at Wave Life Sciences. “Coming out of ObesityWeek®, it is clear there is a strong need for novel non-incretin treatment approaches, and WVE-007 has the potential to disrupt the obesity treatment landscape. The successful clinical translation observed thus far, with robust and durable Activin E reductions, support WVE-007’s potential to induce fat loss, preserve muscle, improve cardiometabolic health, without the class-effects of GLP-1s, and with the advantages of once or twice per year dosing.”

Dr. Bolno added, “We also continue to extend our leadership in the field of RNA editing. In September, we shared data from our ongoing RestorAATion-2 trial that demonstrated WVE-006’s ability to recapitulate an MZ phenotype, including the successful restoration of physiological production of AAT protein at levels needed to prevent lung damage during an acute exacerbation. Building on this clinical success, we advanced WVE-008, our PNPLA3 GalNAc-AIMer for liver disease, as our next RNA editing clinical candidate. With the continued clinical translation of our chemistry across modalities, we are excited to deliver multiple milestones in the coming quarters which have potential to unlock tremendous value with the ultimate goal of bringing transformational medicines to patients in need.”

Recent Business Highlights and Expected Milestones

Obesity

- **WVE-007** is an investigational GalNAc-siRNA, that utilizes Wave’s best-in-class proprietary oligonucleotide chemistry and the company’s **S**tereo**p**ure **i**nterfering **N**ucleic **A**cid (SpiNA) next generation siRNA design. WVE-007 is designed to silence INHBE mRNA, an obesity target with strong evidence from human genetics. Individuals who have a protective loss-of-function variant in one copy of the INHBE gene have a healthier cardiometabolic profile, including less abdominal fat, lower triglycerides, and lower risk of type 2 diabetes and cardiovascular disease.
- At ObesityWeek®, Wave presented preclinical data supporting WVE-007’s potential as a monotherapy treatment, as an add-on to semaglutide, and as a maintenance therapy to prevent rebound weight gain following cessation of GLP-1. In preclinical models, INHBE GalNAc-siRNA led to adipocyte shrinkage, fewer pro-inflammatory macrophages, less fibrosis, and improved insulin sensitivity in adipose tissues, supporting potential for metabolic improvement.
- **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, Activin E, body weight and composition, and biomarkers of metabolic health.
- In October 2025, Wave shared Activin E target engagement data from INLIGHT. Highly significant ($p<0.0001$ for all doses), dose-dependent mean Activin E reductions from baseline were observed at Day 29 (one month post single dose) in the first three cohorts: 85% reduction (Cohort 3), 75% reduction (Cohort 2), 56% reduction (Cohort 1), exceeding levels that led to weight loss and prevention of rebound weight gain following cessation of GLP-1 in preclinical models. Activin E reduction in Cohort 1, the lowest single dose cohort of INLIGHT, was sustained through six months, supporting once or twice a year dosing. WVE-007 was generally safe and well-tolerated.
- INLIGHT is currently ongoing at multiple trial sites, including in the US following clearance of an Investigational New Drug (IND) application. Wave has expanded Cohort 2, Cohort 3, and Cohort 4 (600 mg) to 32 individuals and the independent data monitoring committee approved escalation to a higher dose in Cohort 5.
- **Expected milestones:** Wave expects to deliver multiple clinical data updates from INLIGHT, including body composition and body weight. In the fourth quarter of 2025, Wave expects to deliver three-month follow-up data from Cohort 2 (240 mg), as well as data from Cohort 1 (75 mg). In the first quarter of 2026, Wave expects to deliver six-month follow-up data from Cohort 2 and three-month follow-up data from Cohort 3 (400 mg). In the second quarter of 2026, Wave expects to deliver six-month follow-up data from Cohort 3 and three-month follow-up data from Cohort 4 (600 mg).

AATD (Alpha-1 antitrypsin deficiency)

- **WVE-006** is an investigational 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 volunteer study) 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.
- In September 2025, Wave announced clinical data from the 200 mg single and multidose cohorts and 400 mg single dose cohort of RestorAATion-2 that demonstrate WVE-006 achieved key AATD treatment goals to recapitulate the MZ phenotype. AAT protein exceeded 20 µM during an acute phase response, basal AAT levels reached 13 µM, wild-type M-AAT protein reached 64% of serum AAT, and mutant, Z-AAT was reduced by 60%.
- WVE-006 was generally safe and well tolerated, all adverse events (AEs) were mild to moderate in intensity, and there were no SAEs.
- Dosing is ongoing in the 400 mg multidose cohort with a monthly dosing regimen. Dosing is ongoing in the 600 mg single dose cohort.
- **Expected milestones:** Wave expects to deliver data from the 400 mg multidose cohort in the first quarter of 2026. Wave also expects to deliver single and multidose data from the third and final cohort (600 mg) in 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 the company's proprietary chemistry to achieve potential best-in-class RNA editing and RNAi in a variety of hepatic and extra-hepatic 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. Within RNAi, Wave has shared preclinical data which support that its SpiNA designs enable RNAi-mediated silencing by further improving Ago2 loading and pharmacokinetics, leading to increased potency and durability compared to industry benchmarks.
- **WVE-008:** In October of 2025, Wave announced that it advanced WVE-008, an investigational GalNAc-conjugated RNA editing oligonucleotide (AIMer), as its clinical candidate for PNPLA3 I148M liver disease. There are an estimated nine million homozygous PNPLA3 I148M individuals with liver disease in the U.S. and Europe. These homozygous carriers have a significantly higher risk of liver-related death compared to heterozygous carriers. The PNPLA3 I148M variant is a well-established driver of steatosis, inflammation, ballooning, and fibrosis; however, there are no approved medicines that directly address this biology. Emerging preclinical and clinical data indicate that knocking down PNPLA3 is not an optimal approach as loss of PNPLA3 function inadequately addresses steatosis and fibrosis. To effectively address these manifestations of the disease, Wave uses its novel RNA editing approach. In preclinical studies, Wave has demonstrated that RNA editing with its PNPLA3 GalNAc-AIMer restores functional PNPLA3 protein and decreases lipid accumulation. Wave expects to file a Clinical Trial Application (CTA) for WVE-008 in 2026.
- **Bifunctional single oligonucleotide construct (RNAi+RNA editing):** In October of 2025, Wave shared that it has applied its chemistry optimization with learnings from across its platform to investigate a new modality. This bifunctional single oligonucleotide construct is designed to silence one target while simultaneously editing or upregulating another distinct target. Wave confirmed the ability of its bifunctional single oligonucleotide construct to engage in silencing and editing *in vivo* using a GalNAc-conjugated oligonucleotide that is designed to simultaneously edit UGP2 and silence TTR. In a separate preclinical study, Wave also demonstrated simultaneous upregulation of LDLR and silencing of PCSK9.

Wave shared additional information on its emerging pipeline at its annual Research Day.

DMD (Duchenne muscular dystrophy)

- **WVE-N531** is an investigational 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 generally 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.

HD (Huntington's disease)

- **WVE-003** is a first-in-class, allele-selective investigational 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 feedback from FDA, who recognizes the severity of HD and is 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 IND application for a potentially registrational Phase 2/3 study of WVE-003 in the second half of 2025.

Financial Highlights

- Cash and cash equivalents were $196.2 million as of September 30, 2025, compared to $302.1 million as of December 31, 2024.
- Subsequent to September 30, 2025, $72.1million in ATM proceeds and committed GSK milestones extend Wave's expected cash runway into the second quarter of 2027. By contrast, potential future milestones and other payments to Wave under its GSK collaboration are not included in its cash runway.
- Revenue recognized was $7.6 million for the third quarter of 2025 as compared to ($7.7) million in the prior year quarter.
- Research and development expenses were $45.9 million in the third quarter of 2025 as compared to $41.2 million in the same period in 2024.
- General and administrative expenses were $18.1 million in the third quarter of 2025 as compared to $15.0 million in the same period in 2024.
- Net loss was $53.9 million for the third quarter of 2025 as compared to $61.8 million in the prior year quarter.

Investor Conference Call and Webcast

Wave will host an investor conference call today at 8:30 a.m. ET to review the third 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 obesity, alpha-1 antitrypsin deficiency, 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 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, timing, design, progress, data and announcements related to our clinical trials, including interactions with and feedback from regulators and any potential registrational submissions based on these data; the future performance and results of our programs in clinical trials, including the anticipated therapeutic benefits of such programs and our expectations with respect to how our clinical data may predict success for our future therapeutic candidates and data readouts; the potential commercialization of our programs the potential size of the markets that our therapeutics may address; preclinical activities and programs and their potential to transition into clinical-stage programs, and the timing, progress and announcement of such events; the progress and potential benefits, including the potential achievement of milestones, of collaborations and strategic partnerships; the expected benefits of our stereopure oligonucleotides compared with stereorandom oligonucleotides; the breadth and versatility of our PRISM® drug discovery and development platform; the potential benefits of our RNA editing capability, including our AIMers; the potential benefits of our **S**tereo**p**ure **i**nterfering **N**ucleic **A**cid (SpiNA) next generation siRNA design; 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; our financial performance, including 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. The words "may," "will," "could," "would," "should," "expect," "plan," "anticipate," "intend," "believe," "estimate," "predict," "project," "potential," "continue," "target" and similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words. Any forward-looking statements in this press release are based on management's current expectations and beliefs and are subject to a number of risks, uncertainties and important factors that may cause actual results to differ materially from those indicated by these forward-looking statements as a result of these risks, uncertainties and important factors, including, without limitation, the clinical results and timing of our programs, which may not support further development of our product candidates; actions of regulatory agencies, which may affect the initiation, timing and progress of clinical trials; our effectiveness in managing current and future clinical trials and regulatory processes; the continued development and acceptance of nucleic acid therapeutics as a class of drugs; our ability to demonstrate the therapeutic benefits of our stereopure candidates in clinical trials, including our ability to develop candidates across multiple therapeutic modalities; our ability to obtain, maintain and protect intellectual property; our ability to fund our operations and to raise additional capital as needed; competition from others developing therapies for similar uses; and any impacts on our business as a result of or related to any global economic uncertainty or market disruptions, as well as the other risks and uncertainties described in the section entitled "Risk Factors" in our most recent Annual Report on Form 10-K filed with the Securities and Exchange Commission (SEC), as amended, and in other filings we make with the SEC from time to time. In addition, any forward-looking statements represent our views only as of today and should not be relied upon as representing our views as of any subsequent date. We undertake no obligation, except to the extent required by law, 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)

	September 30, 2025	December 31, 2024
Assets		
Current assets:		
Cash and cash equivalents	$ 196,218	$ 302,078
Accounts receivable	10,000	1,422

Prepaid expenses	6,653	9,544
Other current assets	2,617	7,350
Total current assets	215,488	320,394
Long-term assets:		
Property and equipment, net of accumulated depreciation of $48,755 and $46,329 as of September 30, 2025 and December 31, 2024, respectively	8,196	10,128
Operating lease right-of-use assets	13,876	17,870
Restricted cash	3,796	3,760
Other assets	22	55
Total long-term assets	25,890	31,813
Total assets	$ 241,378	$ 352,207
Liabilities, Series A preferred shares, and shareholders' equity		
Current liabilities:		
Accounts payable	$ 14,490	$ 16,262
Accrued expenses and other current liabilities	16,487	21,081
Current portion of deferred revenue	46,525	65,972
Current portion of operating lease liability	8,394	7,638
Total current liabilities	85,896	110,953
Long-term liabilities:		
Deferred revenue, net of current portion	11,681	6,099
Operating lease liability, net of current portion	11,378	17,766
Total long-term liabilities	23,059	23,865
Total liabilities	$ 108,955	$ 134,818
Series A preferred shares, no par value; 3,901,348 shares issued and outstanding at September 30, 2025 and December 31, 2024	$ 7,874	$ 7,874
Shareholders' equity:		
Ordinary shares, no par value; 160,288,481 and 153,037,286 shares issued and outstanding at September 30, 2025 and December 31, 2024, respectively	$ 1,223,721	$ 1,175,181
Additional paid-in capital	174,075	156,454
Accumulated other comprehensive loss	(190)	(262)
Accumulated deficit	(1,273,057)	(1,121,858)
Total shareholders' equity	$ 124,549	$ 209,515
Total liabilities, Series A preferred shares, and shareholders' equity	$ 241,378	$ 352,207

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

(In thousands, except share and per share amounts)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2025	**2024**	**2025**	**2024**
Revenue	$ 7,608	$ (7,676)	$ 25,482	$ 24,554
Operating expenses:				
Research and development	45,874	41,197	129,965	115,037
General and administrative	18,087	15,042	54,433	42,887
Total operating expenses	63,961	56,239	184,398	157,924
Loss from operations	(56,353)	(63,915)	(158,916)	(133,370)
Other income, net:				
Interest income	2,177	1,798	7,424	6,425
Other income (expense), net	324	337	293	684
Total other income, net	2,501	2,135	7,717	7,109
Loss before income taxes	(53,852)	(61,780)	(151,199)	(126,261)
Income tax benefit (provision)	—	—	—	—
Net loss	$ (53,852)	$ (61,780)	$ (151,199)	$ (126,261)
Net loss per share attributable to ordinary shareholders—basic and diluted	$ (0.32)	$ (0.47)	$ (0.92)	$ (0.97)
Weighted-average ordinary shares used in computing net loss per share attributable to ordinary shareholders—basic and diluted	167,739,208	132,563,467	164,773,525	130,470,603
Other comprehensive income (loss):				
Net loss	$ (53,852)	$ (61,780)	$ (151,199)	$ (126,261)
Foreign currency translation	(29)	120	72	(35)
Comprehensive loss	$ (53,881)	$ (61,660)	$ (151,127)	$ (126,296)

Source: Wave Life Sciences USA, Inc.