# Wave Life Sciences Reports Fourth Quarter and Full Year 2023 Financial Results and Provides Business Update

March 6, 2024

*INHBE lead clinical candidate selected; potential best-in-class treatment for obesity with potent and durable silencing, weight loss with no loss of muscle mass, reduction of visceral fat, and every-six-month or annual subcutaneous dosing; clinical trial initiation expected 1Q 2025*

*Dose escalation ongoing in RestorAATion-1 clinical trial of WVE-006, industry's first-ever RNA editing candidate in the clinic; GalNAc pharmacology translating as expected and proof-of-mechanism data from patients with AATD in RestorAATion-2 on track for 2024*

*Dystrophin data from potentially registrational FORWARD-53 trial of WVE-N531 in DMD on track for 3Q 2024; industry-leading exon skipping levels, muscle tissue concentrations, and myogenic stem cell distribution achieved in Part A clinical trial*

*WVE-003 multi-dose data in HD with extended follow-up on track for 2Q 2024; first-in-class program designed to lower mutant HTT while sparing wild-type HTT*

*Cash and cash equivalents of $200 million as of December 31, 2023; additional $34 million received in 1Q 2024 in a milestone payment from GSK and net proceeds from full exercise of greenshoe option, with runway expected into 4Q 2025; additional anticipated payments under existing collaborations in 2024 and beyond*

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

CAMBRIDGE, Mass., March 06, 2024 (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 fourth quarter and full year ended December 31, 2023, and provided a business update.

"Following a year of execution and tremendous progress, we entered 2024 well-capitalized and uniquely positioned to deliver multiple high-impact clinical data readouts across three different modalities and unlock the broad potential of our pipeline in both rare and prevalent diseases," said Paul Bolno, MD, MBA, President and Chief Executive Officer of Wave Life Sciences. "We are rapidly accelerating the development of our next generation GalNAc-siRNA INHBE program in obesity and are encouraged by the emerging preclinical data, which supports a potential best-in-class profile for this program. These data demonstrate greater knockdown, potency, and durability following a low, single dose, compared to our first generation data, and importantly, demonstrate substantial weight loss with decreases in fat mass and no loss of muscle mass."

Dr. Bolno continued, "In parallel with our efforts to advance our INHBE program, we are continuing to progress our clinical trials. We remain at the forefront of innovation in RNA editing and expect to deliver proof-of-mechanism data for WVE-006 in AATD in 2024. These data would unlock our rapidly advancing pipeline of wholly owned editing candidates. Beyond editing, we remain on track to deliver multidose data for our novel, allele-selective HD program in the second quarter and potentially registrational data from our FORWARD-53 trial in DMD in the third quarter. The coming months have the potential to be truly transformative for Wave."

**Recent Business Highlights**

***AATD and AIMer pipeline (RNA editing)***

- **Dose escalation in RestorAATion-1 clinical trial ongoing; clinical program investigating WVE-006 as a first- and best-in-class treatment for alpha-1 antitrypsin deficiency (AATD).**
  - WVE-006 is a GalNAc-conjugated, subcutaneously delivered, RNA editing oligonucleotide that is uniquely designed to address AATD-related lung disease, liver disease, or both.
  - The RestorAATion clinical program is evaluating WVE-006 for AATD and includes healthy volunteers (RestorAATion-1), as well as patients who have the homozygous Pi*ZZ mutation (RestorAATion-2). It is designed to provide an efficient path to proof-of-mechanism as measured by restoration of wild-type alpha-1 antitrypsin (M-AAT) protein in serum.
  - In the fourth quarter of 2023, Wave initiated dosing in RestorAATion-1, which resulted in a $20 million milestone payment from GSK. Dose escalation is ongoing and WVE-006's pharmacology in healthy volunteers is translating as expected.
  - Beyond WVE-006, Wave is advancing a pipeline of wholly owned RNA editing therapeutics across a range of high-impact GalNAc-hepatic and extra-hepatic targets. Utilizing its proprietary "edit-verse," which is powered by genetic datasets and deep learning models, Wave has identified several new RNA editing targets that leverage easily accessible biomarkers, offer efficient paths to proof-of-concept in humans, and represent meaningful commercial opportunities. Wave plans to share new preclinical data for its advancing RNA editing programs in 2024.
  - **Expected upcoming milestone:** Wave expects to deliver proof-of-mechanism data from RestorAATion-2 in patients with AATD in 2024.

***Obesity (siRNA)***

- **Selected lead clinical candidate for INHBE program with a potentially best-in-class profile for obesity and accelerated clinical development timeline.**
  - Today, Wave announced the selection of its INHBE lead clinical candidate, ahead of prior expectations, and now

plans to submit a clinical trial application for its INHBE program as early as year-end 2024.
    - Wave's INHBE clinical candidate is a GalNAc-small interfering RNA (siRNA) that utilizes Wave's next generation siRNA format and is designed to silence the INHBE (Inhibin βE) gene through RNA knockdown with a goal of inducing lipolysis (fat-burning) while preserving muscle mass to restore and maintain a healthy metabolic profile.
    - INHBE loss-of-function (LoF) heterozygous human carriers have a favorable cardiometabolic profile, including reduced abdominal obesity and reduced odds of type 2 diabetes and coronary artery disease. Silencing INHBE is expected to recapitulate the cardiometabolic profile of these LoF carriers and may also address the limitations of GLP-1s as a monotherapy or be used as an adjunct therapy with GLP-1s.
    - Key data highlights from preclinical mouse models include:
        - Highly potent silencing (ED50 < 1mg/kg)
        - Durable silencing following one, low-single-digit dose supporting every-six-month or annual subcutaneous dosing
        - Weight loss with no loss of muscle mass
        - Reduction in fat mass with preferential effects on visceral fat, consistent with the profile of INHBE LoF carriers in human genetics
    - **Expected upcoming milestone:** Wave expects to initiate a clinical trial for its INHBE candidate in the first quarter of 2025.

***DMD (exon skipping)***

- **Advancing FORWARD-53 clinical trial; multiple presentations at MDA Clinical & Scientific Conference highlight differentiated profile including industry-leading exon-skipping, muscle tissue concentrations and myogenic stem cell distribution.**
    - Wave's WVE-N531 program for boys with Duchenne muscular dystrophy (DMD) amenable to exon 53 skipping is designed to induce production of endogenous, functional dystrophin protein.
    - This week, at the 2024 MDA Clinical & Scientific Conference, Wave presented multiple posters highlighting WVE-N531. These posters featured data from Part A of Wave's WVE-N531 trial, demonstrating industry-leading exon skipping levels of 53% and muscle tissue concentrations of 42 µg/g (42,000 ng/g), as well as stem cell distribution in all study participants.
    - WVE-N531 is currently being evaluated in the ongoing FORWARD-53 clinical trial of 11 boys with DMD, which is powered to evaluate functional, endogenous dystrophin expression following 24 and 48 weeks of 10 mg/kg dosing administered every-other-week. The primary endpoint is dystrophin protein levels, and the trial will also evaluate pharmacokinetics, digital and functional endpoints, and safety and tolerability.
    - Pending positive results from the FORWARD-53 trial, the company is planning to advance a broader DMD pipeline with PN-modified oligonucleotides for skipping other exons, with the goal of providing new treatment options for a larger population of boys with DMD.
    - **Expected upcoming milestone:** Wave expects to deliver data, including dystrophin protein expression from muscle biopsies at 24 weeks, in the third quarter of 2024.

***HD (antisense silencing)***

- **WVE-003 and wild-type HTT sparing highlighted at annual CHDI Conference.**
    - WVE-003 is a first-in-class investigational allele-selective Huntington's disease (HD) therapeutic designed to reduce mutant huntingtin (mHTT) protein while also sparing healthy wild-type huntingtin (wtHTT) protein. Due to the significance of wtHTT function for the health of the central nervous system and the potential for mHTT to disrupt wtHTT function, selectively lowering mHTT while preserving wtHTT protein expression and function may offer advantages over nonselective HTT-lowering approaches for the treatment of HDrecent publication.
    - WVE-003 has demonstrated single-dose reductions in mean mHTT in cerebrospinal fluid of 35% compared to placebo, with preservation of wtHTT, as previously shared in September 2022.
    - The ongoing multi-dose portion of the SELECT-HD clinical trial is evaluating a cohort of 24 patients with HD receiving 30 mg doses of WVE-003 administered every eight weeks.
    - Data from the ongoing SELECT-HD clinical trial will form the basis for decision making for Wave's advancement of this program, including supporting an opt-in package for Takeda.
    - **Expected milestone:** Wave expects to report data from the 30 mg multi-dose cohort with extended follow-up, along with all single-dose data, in the second quarter of 2024.

**Financial Highlights**

- Cash and cash equivalents were $200.4 million as of December 31, 2023, compared to $88.5 million as of December 31, 2022. The increase in cash year-over-year is primarily due to the $170.0 million of cash received from the GSK Collaboration and the $93.6 million in net proceeds from the December 2023 Offering, partially offset by the net loss for the year. Subsequent to December 31, 2023, Wave received $20 million in a milestone payment from GSK and $14.0 million in net proceeds from the full exercise of the greenshoe option related to the December 2023 financing.
- Wave expects that its current cash and cash equivalents will be sufficient to fund operations into the fourth quarter of 2025. Potential future milestone and other payments under the company's GSK and Takeda collaborations are not included in its cash runway.
- Revenue was $29.1 million for the fourth quarter of 2023 as compared to $1.2 million in the prior year quarter. Revenue was $113.3 million in 2023 as compared to $3.6 million in 2022. The increase in revenue was due to revenue earned from

the company's collaborations with both GSK and Takeda.

- Research and development expenses were $34.1 million in the fourth quarter of 2023 as compared to $31.1 million in the same period in 2022. Research and development expenses for the full year were $130.0 million in 2023, as compared to $115.9 million in 2022.
- General and administrative expenses were $13.7 million in the fourth quarter of both 2023 and 2022. General and administrative expenses for the full year were $51.3 million in 2023, as compared to $50.5 million in 2022.
- Net loss was $16.3 million for the fourth quarter of 2023 as compared to $43.7 million in the prior year quarter. Net loss for the full year was $57.5 million for 2023 as compared to $161.8 million in 2022. Net loss significantly improved over the prior year due to the substantial revenue earned from collaboration partners in 2023.

**Investor Conference Call and Webcast**

Wave will host an investor conference call today at 8:30 a.m. ET to review the fourth quarter and full year 2023 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 following audio-conferencing link: available here. Once registered, participants will receive the dial-in information. 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 prevalent 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 Duchenne muscular dystrophy, Alpha-1 antitrypsin deficiency and Huntington's disease, as well as a preclinical program in obesity. 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 announcement of such events; the protocol, design and endpoints of our clinical trials; the future performance and results of our programs in clinical trials; future preclinical activities and programs; regulatory submissions; the progress and potential benefits of our collaborations; the potential achievement of milestones under our collaborations and receipt of cash payments therefor; the potential of our preclinical data to predict the behavior of our compounds in humans; our identification and expected timing of future product candidates and their therapeutic potential; the anticipated benefits of our therapeutic candidates and pipeline compared to our competitors; our ability to design compounds using various modalities and the anticipated benefits of that approach; the breadth and versatility of our PRISM drug discovery and development platform; the expected benefits of our stereopure oligonucleotides compared with stereorandom oligonucleotides; 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; the potential benefits of our GalNAc-conjugated siRNA program targeting INHBE; the potential benefits that our "edit-verse" may provide us to identify new RNA editing targets; the status and progress of our programs relative to potential competitors; anticipated benefits of our proprietary manufacturing processes and our internal manufacturing capabilities; the benefits of RNA medicines generally; the strength of our intellectual property and the data that support our IP; the anticipated duration of our cash runway; 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 adaptive trial designs, which may affect the initiation, timing and progress of clinical trials; our effectiveness in managing regulatory interactions and future clinical trials; the effectiveness of PRISM; 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; 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.

**WAVE LIFE SCIENCES LTD.**
**UNAUDITED CONSOLIDATED BALANCE SHEETS**

*(In thousands, except share amounts)*

| | December 31, 2023 | December 31, 2022 |
|---|---|---|
| **Assets** | | |
| Current assets: | | |
| Cash and cash equivalents | $ 200,351 | $ 88,497 |
| Accounts receivable | 21,086 | — |
| Prepaid expenses | 9,912 | 7,932 |
| Other current assets | 4,024 | 2,108 |
| Total current assets | 235,373 | 98,537 |
| Long-term assets: | | |
| Property and equipment, net of accumulated depreciation of $42,709 and $37,846 as of December 31, 2023 and 2022, respectively | 13,084 | 17,284 |
| Operating lease right-of-use assets | 22,637 | 26,843 |

| | | |
|---|---|---|
| Restricted cash | 3,699 | 3,660 |
| Other assets | 156 | 62 |
| Total long-term assets | 39,576 | 47,849 |
| Total assets | $ 274,949 | $ 146,386 |
| **Liabilities, Series A preferred shares and shareholders' equity (deficit)** | | |
| Current liabilities: | | |
| Accounts payable | $ 12,839 | $ 16,915 |
| Accrued expenses and other current liabilities | 16,828 | 17,552 |
| Current portion of deferred revenue | 150,059 | 31,558 |
| Current portion of operating lease liability | 6,714 | 5,496 |
| Total current liabilities | 186,440 | 71,521 |
| Long-term liabilities: | | |
| Deferred revenue, net of current portion | 15,601 | 79,774 |
| Operating lease liability, net of current portion | 25,404 | 32,118 |
| Other liabilities | — | 190 |
| Total long-term liabilities | 41,005 | 112,082 |
| Total liabilities | $ 227,445 | $ 183,603 |
| Series A preferred shares, no par value; 3,901,348 shares issued and outstanding at December 31, 2023 and 2022 | $ 7,874 | $ 7,874 |
| Shareholders' equity (deficit): | | |
| Ordinary shares, no par value; 119,162,234 and 86,924,643 shares issued and outstanding at December 31, 2023 and 2022, respectively | $ 935,367 | $ 802,833 |
| Additional paid-in capital | 129,237 | 119,442 |
| Accumulated other comprehensive loss | (124) | (29) |
| Accumulated deficit | (1,024,850) | (967,337) |
| Total shareholders' equity (deficit) | 39,630 | (45,091) |
| Total liabilities, Series A preferred shares and shareholders' equity (deficit) | $ 274,949 | $ 146,386 |

**WAVE LIFE SCIENCES LTD.**
**UNAUDITED CONSOLIDATED STATEMENTS OF OPERATIONS AND COMPREHENSIVE INCOME (LOSS)**

*(In thousands, except share and per share amounts)*

| | Three Months Ended December 31, | | Twelve Months Ended December 31, | |
|---|---|---|---|---|
| | 2023 | 2022 | 2023 | 2022 |
| Revenue | $ 29,056 | $ 1,239 | $ 113,305 | $ 3,649 |
| Operating expenses: | | | | |
| Research and development | 34,074 | 31,078 | 130,009 | 115,856 |
| General and administrative | 13,664 | 13,724 | 51,292 | 50,513 |
| Total operating expenses | 47,738 | 44,802 | 181,301 | 166,369 |
| Loss from operations | (18,682) | (43,563) | (67,996) | (162,720) |
| Other income, net: | | | | |
| Dividend income and interest income, net | 1,844 | 825 | 7,928 | 1,571 |
| Other income (expense), net | 582 | (290) | 1,878 | 7 |
| Total other income, net | 2,426 | 535 | 9,806 | 1,578 |
| Loss before income taxes | (16,256) | (43,028) | (58,190) | (161,142) |
| Income tax benefit (provision) | — | (681) | 677 | (681) |
| Net loss | $ (16,256) | $ (43,709) | $ (57,513) | $ (161,823) |
| Net loss per share attributable to ordinary shareholders—basic and diluted | $ (0.15) | $ (0.47) | $ (0.54) | $ (2.05) |
| Weighted-average ordinary shares used in computing net loss per share attributable to ordinary shareholders—basic and diluted | 109,627,549 | 93,993,638 | 106,097,268 | 78,855,810 |
| Other comprehensive loss: | | | | |
| Net loss | $ (16,256) | $ (43,709) | $ (57,513) | $ (161,823) |
| Foreign currency translation | 58 | 94 | (95) | (210) |
| Comprehensive loss | $ (16,198) | $ (43,615) | $ (57,608) | $ (162,033) |

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