# Wave Life Sciences Reports First Quarter 2023 Financial Results and Provides Business Update

May 3, 2023

*Emerging leader in RNA medicines with multi-modal discovery and development platform and first-in-class RNA editing programs*

*Rapidly advancing toward 2023 CTA submissions and first-in-human study for WVE-006, the industry's first RNA editing clinical candidate*

*Planning for potentially registrational Phase 2 clinical study for WVE-N531 to assess functional dystrophin protein restoration in DMD patients, following best-in-class exon-skipping data reported in December 2022*

*Preparing broad suite of first- and best-in-class medicines enabled by multi-modal RNA medicine platform and proprietary genetic insights from GSK collaboration; investor event anticipated in 3Q 2023*

*Cash and cash equivalents of $207.6 million as of March 31, 2023, with runway expected into 2025, plus potential milestone payments from GSK collaboration in 2023 and beyond*

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

CAMBRIDGE, Mass., May 03, 2023 (GLOBE NEWSWIRE) -- Wave Life Sciences Ltd. (Nasdaq: WVE), a clinical-stage RNA medicines company committed to delivering life-changing treatments for people battling devastating diseases, today announced financial results for the first quarter ended March 31, 2023 and provided a business update.

"The first quarter of 2023 was marked by the launch of our transformational collaboration with GSK, which we believe will allow us to build substantial patient and shareholder value by expanding and accelerating our pipeline with capital, clinical capability, and proprietary genetic insights. This collaboration, which provided $170 million in upfront cash and equity, has the potential to provide additional cash milestones in 2023 and beyond. We are expeditiously progressing the industry's first RNA editing candidate, WVE-006, toward CTA submissions for alpha-1 antitrypsin deficiency, with the goal of early clinical proof-of-concept via measurement of validated serum surrogate markers in a clinical trial. In the first quarter, we also prepared for the launch of a potentially registrational Phase 2 study of WVE-N531 for boys with DMD, which is a program we believe may provide an important therapeutic option for them," said Paul Bolno, MD, MBA, President and Chief Executive Officer of Wave Life Sciences. "We are well-capitalized through our existing cash and potential near-term milestones to deliver on a steady cadence of clinical data across 2023 and 2024. Additionally, using our clinically validated multi-modal RNA medicines platform, we are preparing the next set of development programs that leverage our unique RNA editing, splicing, and knockdown capabilities. In the third quarter of this year, we plan to hold an investor event, during which we will demonstrate how we are continuing to extend our leadership in RNA editing and share preclinical data on new programs."

**Recent Business Highlights**

- **Presented WVE-N531 clinical data at MDA Conference; progressing WVE-N531 to Phase 2 clinical trial to evaluate functional dystrophin protein production in boys with Duchenne muscular dystrophy (DMD) amenable to exon 53 skipping.** In March 2023, at the Muscular Dystrophy Association (MDA) Clinical and Scientific Conference, Wave presented encouraging data from the initial cohort of the proof-of-concept open-label study of WVE-N531 to neuromuscular disease clinicians for the first time. Data for WVE-N531 included observation of best-in-class exon skipping and high muscle concentrations, while appearing safe and well-tolerated. A potentially registrational Phase 2 trial of WVE-N531 is planned, which will be powered to evaluate functional dystrophin expression following 24 and 48 weeks of biweekly dosing of WVE-N531. The primary endpoint will be dystrophin protein levels, and the study will also evaluate safety and tolerability, pharmacokinetics, and functional endpoints. Data are expected in 2024. If successful, WVE-N531 has potential to become a near-term wholly-owned commercial opportunity for Wave and would enable accelerated development of additional exon skipping candidates for other mutations.

- **Collaboration activities with GSK underway.** Wave's strategic collaboration with GSK, to advance transformative RNA medicines using Wave's multi-modal RNA platform, became effective in January 2023 and multiple target validation programs are already underway. The collaboration is designed to provide multiple value drivers to Wave, including maximizing the commercial opportunity for WVE-006 in alpha-1 antitrypsin deficiency (AATD), expanding Wave's pipeline with new targets leveraging unique genetic insights from GSK, and continuing opportunities to strengthen Wave's balance sheet.

- **Advancing WVE-006, a first-in-class RNA editing therapeutic for AATD, towards CTA submissions.** At multiple scientific conferences in the first quarter of 2023, Wave highlighted the preclinical data supporting WVE-006, its GalNAc-conjugated candidate for AATD. WVE-006 is Wave's first A-to-I(G) RNA base editing ("AIMer") development candidate, which is also first-in-class in AATD, and is uniquely designed for restoration of both healthy hepatic and pulmonary function with the opportunity for reversibility and a favorable safety profile. IND enabling studies for WVE-006 continue to advance and Wave is on track to submit clinical trial applications (CTAs) in the second half of 2023.

- **Presented leading RNA editing capability at Gordon Research Conference.** In March 2023, at the RNA Editing 2023 Gordon Research Conference, Wave presented an overview of its therapeutic base editing platform, including its novel base modifications and improvements in editing activity with optimized designs.

**Recent Scientific Publications**

- In April 2023, preclinical data for the company's novel siRNA formats were published in the journal of Nucleic Acids Research. The preclinical data demonstrated unprecedented, best-in-class Ago2 loading following administration of a single subcutaneous GalNAc-siRNA dose, leading to improved potency and durability *in vivo* versus comparator siRNA formats. Wave's RNAi capability is one of multiple modalities being advanced through the strategic research collaboration with GSK. All of Wave's publications can be viewed here.

**Anticipated Upcoming Milestones and Events**

WVE-N531 for DMD:

- Initiate dosing in Part B of WVE-N531 potentially registrational Phase 2 clinical trial in 2023
- Deliver data from Part B in 2024

WVE-006 for AATD:

- Submit CTAs for first-in-human study in 2H 2023

WVE-003 for HD:

- Deliver additional single-dose and multi-dose biomarker and safety clinical data in 2H 2023
- The update in expected timing for HD single-dose clinical data is due to a publicly announced cyber-attack that took place at Wave's mHTT assay vendor in April 2023. No Wave data or patient samples were impacted by the attack and Wave remains in close contact with the vendor as they address this issue.

WVE-004 for ALS/FTD:

- Deliver additional single- and multi-dose biomarker and safety clinical data in 1H 2023

Platform and Pipeline:

- Anticipate virtual investor event to be held in the third quarter of 2023 during which Wave will demonstrate how it is continuing to extend its leadership in RNA editing and share preclinical data on new wholly-owned programs
- Advance collaboration activities with GSK, with potential for additional cash inflows in 2023 and beyond

**First Quarter 2023 Financial Results**

Wave reported a net loss of $27.4 million in the first quarter of 2023, as compared to $37.8 million in the same period in 2022. The decrease in net loss year-over-year was primarily driven by revenue earned under the company's collaboration with GSK, which became effective January 27, 2023. Revenue earned under the GSK and Takeda collaborations in the first quarter of 2023 was $12.9 million. During the first quarter of 2022, revenue of $1.8 million was primarily earned under the Takeda collaboration.

Research and development expenses were $31.0 million in the first quarter of 2023, as compared to $27.5 million in the same period in 2022. The increase in research and development expenses was primarily due to increased external expenses related to Wave's clinical programs, as well as compensation-related expenses driven by growth to support the company's programs.

General and administrative expenses were $12.2 million in the first quarter of 2023, as compared to $12.4 million in the same period in 2022, primarily due to a decrease in compensation-related expenses.

As of March 31, 2023, Wave had $207.6 million in cash and cash equivalents, as compared to $88.5 million as of December 31, 2022. The company expects that its current cash and cash equivalents will be sufficient to fund operations into 2025.

**Investor Conference Call and Webcast**
Wave will host an investor conference call today at 8:30 a.m. ET to review first quarter 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-and-presentations. 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 clinical-stage RNA medicines company committed to delivering life-changing treatments for people battling devastating diseases. Wave aspires to develop best-in-class medicines across multiple therapeutic modalities using PRISM, the company's proprietary discovery and drug development platform that enables the precise design, optimization, and production of stereopure oligonucleotides. Driven by a resolute sense of urgency, the Wave team is targeting a broad range of genetically defined diseases so that patients and families may realize a brighter future. To find out more, please visit www.wavelifesciences.com and follow Wave on Twitter @WaveLifeSci.

**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 of data and completion of our clinical trials, 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 compared to others; our ability to design compounds using multiple modalities and the anticipated benefits of that approach; the breadth and versatility of PRISM; 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 status and progress of our programs relative to potential

competitors; anticipated benefits of our proprietary manufacturing processes and our internal manufacturing capabilities; the benefit of nucleic acid therapeutics 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 any potential global macro events beyond our control 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)*

| | March 31, 2023 | December 31, 2022 |
|---|---|---|
| **Assets** | | |
| Current assets: | | |
| Cash and cash equivalents | $ 207,562 | $ 88,497 |
| Prepaid expenses | 9,231 | 7,932 |
| Other current assets | 2,798 | 2,108 |
| Total current assets | 219,591 | 98,537 |
| Long-term assets: | | |
| Property and equipment, net of accumulated depreciation of $39,197 and $37,846 as of March 31, 2023 and December 31, 2022, respectively | 16,005 | 17,284 |
| Operating lease right-of-use assets | 25,838 | 26,843 |
| Restricted cash | 4,660 | 3,660 |
| Other assets | 1,176 | 62 |
| Total long-term assets | 47,679 | 47,849 |
| Total assets | $ 267,270 | $ 146,386 |
| **Liabilities, Series A preferred shares and shareholders’ equity (deficit)** | | |
| Current liabilities: | | |
| Accounts payable | $ 11,906 | $ 16,915 |
| Accrued expenses and other current liabilities | 7,622 | 17,552 |
| Current portion of deferred revenue | 106,960 | 31,558 |
| Current portion of operating lease liability | 6,078 | 5,496 |
| Total current liabilities | 132,566 | 71,521 |
| Long-term liabilities: | | |
| Deferred revenue, net of current portion | 130,820 | 79,774 |
| Operating lease liability, net of current portion | 30,534 | 32,118 |
| Other liabilities | 190 | 190 |
| Total long-term liabilities | 161,544 | 112,082 |
| Total liabilities | $ 294,110 | $ 183,603 |
| Series A preferred shares, no par value; 3,901,348 shares issued and outstanding at March 31, 2023 and December 31, 2022 | $ 7,874 | $ 7,874 |
| Shareholders’ equity (deficit): | | |
| Ordinary shares, no par value; 98,104,844 and 86,924,643 shares issued and outstanding at March 31, 2023 and December 31, 2022, respectively | $ 837,886 | $ 802,833 |
| Additional paid-in capital | 122,192 | 119,442 |
| Accumulated other comprehensive income (loss) | (50) | (29) |
| Accumulated deficit | (994,742) | (967,337) |
| Total shareholders’ equity (deficit) | $ (34,714) | $ (45,091) |
| Total liabilities, Series A preferred shares and shareholders’ equity (deficit) | $ 267,270 | $ 146,386 |

**WAVE LIFE SCIENCES LTD.**
**UNAUDITED CONSOLIDATED STATEMENTS OF OPERATIONS AND COMPREHENSIVE LOSS**
*(In thousands, except share and per share amounts)*

| | Three Months Ended March 31, | |
|---|---|---|
| | 2023 | 2022 |

| | | |
|---|---|---|
| Revenue | $ 12,929 | $ 1,750 |
| Operating expenses: | | |
| Research and development | 30,979 | 27,470 |
| General and administrative | 12,235 | 12,374 |
| Total operating expenses | 43,214 | 39,844 |
| Loss from operations | (30,285) | (38,094) |
| Other income, net: | | |
| Dividend income and interest income, net | 1,873 | 26 |
| Other income, net | 1,007 | 254 |
| Total other income, net | 2,880 | 280 |
| Loss before income taxes | (27,405) | (37,814) |
| Income tax provision | — | — |
| Net loss | $ (27,405) | $ (37,814) |
| Net loss per share attributable to ordinary shareholders—basic and diluted | $ (0.27) | $ (0.62) |
| Weighted-average ordinary shares used in computing net loss per share attributable to ordinary shareholders—basic and diluted | 102,056,712 | 60,516,616 |
| | | |
| Other comprehensive loss: | | |
| Net loss | $ (27,405) | $ (37,814) |
| Foreign currency translation | (21) | (86) |
| Comprehensive loss | $ (27,426) | $ (37,900) |

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