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

November 9, 2023

Initiated RestorAATion clinical program for WVE-006, industry's first-ever RNA editing clinical candidate, following approval of CTAs; dosing on track for 4Q 2023 and first-in-human proof-of-mechanism data anticipated in 2024

On track to deliver dystrophin data from potentially registrational FORWARD-53 trial of WVE-N531 in 2024

Enrollment complete in 30 mg multi-dose cohort of SELECT-HD with clinical data for decision-making on track for 2Q 2024; achieved milestone from Takeda collaboration for NHP study that further reinforces productive distribution of WVE-003 to deep brain regions

Presented preclinical proof-of-concept data for new, wholly owned GalNAc-siRNA INHBE program for metabolic disorders, including obesity; leads identified and clinical candidate selection expected in 4Q 2024

Cash and cash equivalents of $140 million as of September 30, 2023, plus $7 million milestone payment from Takeda collaboration received in 4Q, with runway expected into 2025; additional potential near-term milestone payments from GSK collaboration in 2023 and beyond

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

CAMBRIDGE, Mass., Nov. 09, 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 third quarter ended September 30, 2023, and provided a business update.

"The team has made tremendous progress during the past quarter as we solidified our leadership in RNA editing through the initiation of our clinical trial evaluating the first-ever RNA editing therapeutic, WVE-006. Additionally this quarter, we advanced our WVE-N531 (DMD) and WVE-003 (HD) clinical development programs and expanded our RNA therapeutic capabilities beyond rare diseases to common diseases with the announcement of our first GalNAc-conjugated siRNA program targeting INHBE to treat metabolic disorders, including obesity," said Paul Bolno, MD, MBA, President and Chief Executive Officer of Wave Life Sciences. "As we shared during our recent R&D Day, we will sustain our leadership in RNA editing by building a pipeline of wholly owned editing candidates, each of which will have efficient paths to clinical proof-of-concept and represent significant commercial opportunities. With multiple, high impact clinical data readouts expected over the course of 2024 and plans to expeditiously deliver on a growing pipeline of RNA-targeting modalities, Wave is truly at an exciting inflection point."

Recent Business Highlights

- **Initiated RestorAATion clinical program investigating WVE-006 as a treatment for alpha-1 antitrypsin deficiency (AATD).** Wave recently initiated RestorAATion following approval of multiple clinical trial applications (CTAs). WVE-006 is uniquely designed to address AATD-related lung disease, liver disease, or both. The RestorAATion clinical program includes healthy volunteers (RestorAATion-1), as well as individuals with AATD who have the homozygous PiZZ mutation (RestorAATion-2) and 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. Wave expects to initiate dosing with WVE-006 in healthy volunteers in the fourth quarter of 2023 and deliver proof-of-mechanism data in individuals with AATD in 2024.

- **Announced plans to advance a wholly owned pipeline of RNA editing candidates.** Wave is utilizing its proprietary "edit-verse," which is powered by genetic datasets and deep learning models and designed to identify new RNA editing targets and edit sites. These targets leverage easily accessible biomarkers, offer efficient paths to proof-of-concept in humans, and represent meaningful commercial opportunities. Wave demonstrated *in vivo* and *in vitro* proof-of-concept data on several of these new targets, achieving at least 2-fold mRNA upregulation in liver and kidney targets and more than 60% mRNA correction in liver and lung targets.

- **Announced new, wholly owned GalNAc-conjugated siRNA program targeting INHBE for metabolic disorders, including obesity, along with preclinical *in vivo* proof-of-concept data.** INHBE loss-of-function heterozygous human carriers exhibit a healthy metabolic profile, including reduced waist-to-hip ratio and reduced odds of type 2 diabetes and coronary artery disease, and INHBE reduction of 50% or greater is expected to restore a healthy metabolic profile. Wave presented *in vivo* data at its recent R&D Day demonstrating INHBE silencing well beyond the 50% therapeutic threshold, which led to substantially lower body weight and reduction of visceral fat in diet-induced obesity mice as compared to control. These are the first *in vivo* data to demonstrate INHBE silencing is consistent with the phenotypes of heterozygous loss-of-function carriers. Wave has identified potent and highly specific leads and expects to select an INHBE clinical candidate in the fourth quarter of 2024. The INHBE program is Wave's first wholly owned program to emerge from its collaboration with GSK.

- **Advancing potentially registrational Phase 2 FORWARD-53 clinical trial for WVE-N531.** Wave remains on track to initiate dosing in FORWARD-53 in 2023 and enroll up to 10 boys in the trial. The clinical trial is powered to evaluate functional, endogenous dystrophin expression following 24 and 48 weeks of bi-weekly dosing. The primary endpoint will be dystrophin protein levels, and the trial will also evaluate pharmacokinetics, digital and functional endpoints, and safety and tolerability. Pending positive results from this trial, the company is planning to advance a broader DMD pipeline with

PN-modified splicing oligonucleotides for skipping other exons, with the goal of providing new treatment options for a larger population of boys with DMD. Data from FORWARD-53 are expected in 2024.

- **Delivered first clinical data in DMD demonstrating uptake in satellite cells after three biweekly doses**. At Wave's annual R&D Day held in September 2023, the company highlighted clinical data from Part A of the Phase 1b/2a trial indicating that WVE-N531 was present in myogenic satellite cells, which is important for potential muscle regeneration. These are the first clinical data in DMD to demonstrate uptake in satellite cells at this early time point and further support the potential differentiation of WVE-N531 from other therapeutics, including gene therapies.

- **Achievement of a non-clinical milestone under Takeda collaboration; NHP data supports broad distribution of WVE-003 in CNS.** WVE-003 is the most advanced investigational HD therapeutic designed to reduce mutant huntingtin (mHTT) protein while also sparing healthy, wild-type huntingtin (wtHTT) protein, and previously demonstrated single-dose reductions in mean CSF mHTT of 35% as compared to placebo, with preservation of wtHTT, in September 2022. In the third quarter of 2023, Wave achieved a milestone in its collaboration with Takeda, which pertained to the positive results from a non-clinical study of WVE-003 in non-human primates (NHPs) and resulted in a payment of $7 million to Wave in the fourth quarter of 2023. This study showed significant tissue exposure levels of WVE-003 in the deep brain regions, including striatum, and bolstered Wave's existing datasets that confirm the ability of its oligonucleotides to distribute to the areas of the CNS important for HD.

- **Completed enrollment in 30 mg multi-dose cohort of Phase 1b/2a SELECT-HD clinical trial for WVE-003 – the most advanced allele-selective investigational therapeutic for individuals with HD.** Wave recently completed enrollment in the 30 mg multi-dose cohort of the SELECT-HD clinical trial, which is evaluating doses administered every eight weeks. Having rolled over patients from the single-dose cohort and fully enrolled the 30 mg multi-dose cohort, Wave will now evaluate the completed single-dose and multi-dose cohorts simultaneously. 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. These data are expected to enable decision making on the program and support the company's opt-in package for Takeda.

Anticipated Upcoming Milestones

WVE-006 for AATD:

- Initiate dosing in healthy volunteers in RestorAATion clinical program in 4Q 2023
- Deliver proof-of-mechanism data from RestorAATion clinical program in 2024

WVE-N531 for DMD:

- Initiate dosing in potentially registrational FORWARD-53 Phase 2 clinical trial in 2023
- Deliver data from FORWARD-53 clinical trial in 2024

WVE-003 for HD:

- Deliver data from 30 mg multi-dose cohort with extended follow-up, along with all single-dose data, in 2Q 2024

INHBE program for metabolic disorders, including obesity

- Select a clinical candidate in 4Q 2024

Platform and Pipeline:

- Advance collaboration activities with GSK, with potential for additional cash inflows in 2023 and beyond
- Select five new clinical candidates by year-end 2025, including INHBE

Third Quarter 2023 Financial Results

Wave reported a net income of $7.3 million in the third quarter of 2023, as compared to a net loss of $39.0 million in the same period in 2022. The year-over-year change was primarily driven by the $48.9 million increase in revenue recognized under its collaborations. Under the Takeda collaboration, Wave earned $7.0 million dollars for the achievement of a WVE-003 non-clinical milestone. Additionally, under the Takeda collaboration, Wave recognized $28.0 million relating to the discontinuation of WVE-004 and under the GSK Collaboration, recognized $14.3 million.

Research and development expenses were $31.6 million in the third quarter of 2023, as compared to $27.6 million in the same period in 2022. The increase in research and development expenses was primarily driven by increased external expenses related to all three of Wave's clinical programs.

General and administrative expenses were $13.1 million in the third quarter of 2023, as compared to $11.6 million in the same period in 2022. The increase was primarily due to increased professional and consulting expenses as well as other general expenses.

As of September 30, 2023, Wave had $139.9 million in cash and cash equivalents, as compared to $88.5 million as of December 31, 2022. Subsequent to the quarter end, Wave received the $7.0 million for the achievement of the previously discussed milestone. 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 third 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 X (formerly 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, including 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 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 potential benefits of our GalNAc-conjugated siRNA program targeting INHBE, the potential benefits that our "edit-verse" map may offer 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 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)

	September 30, 2023	December 31, 2022
Assets		
Current assets:		
Cash and cash equivalents	$ 139,942	$ 88,497
Accounts receivable	7,000	—
Prepaid expenses	7,514	7,932
Other current assets	4,116	2,108
Total current assets	158,572	98,537
Long-term assets:		
Property and equipment, net of accumulated depreciation of $41,596 and $37,846 as of September 30, 2023 and December 31, 2022, respectively	13,770	17,284
Operating lease right-of-use assets	23,738	26,843
Restricted cash	3,683	3,660
Other assets	155	62
Total long-term assets	41,346	47,849
Total assets	$ 199,918	$ 146,386
Liabilities, Series A preferred shares and shareholders' equity (deficit)		
Current liabilities:		
Accounts payable	$ 12,608	$ 16,915
Accrued expenses and other current liabilities	12,624	17,552
Current portion of deferred revenue	68,251	31,558
Current portion of operating lease liability	6,497	5,496
Total current liabilities	99,980	71,521
Long-term liabilities:		
Deferred revenue, net of current portion	105,380	79,774
Operating lease liability, net of current portion	27,170	32,118
Other liabilities	—	190
Total long-term liabilities	132,550	112,082

Total liabilities	$ 232,530	$ 183,603
Series A preferred shares, no par value; 3,901,348 shares issued and outstanding at September 30, 2023 and December 31, 2022	$ 7,874	$ 7,874
Shareholders' equity (deficit):		
Ordinary shares, no par value; 99,011,901 and 86,924,643 shares issued and outstanding at September 30, 2023 and December 31, 2022, respectively	$ 841,405	$ 802,833
Additional paid-in capital	126,885	119,442
Accumulated other comprehensive loss	(182)	(29)
Accumulated deficit	(1,008,594)	(967,337)
Total shareholders' deficit	$ (40,486)	$ (45,091)
Total liabilities, Series A preferred shares and shareholders' deficit	$ 199,918	$ 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 September 30,		Nine Months Ended September 30,	
	2023	2022	2023	2022
Revenue	$ 49,214	$ 285	$ 84,249	$ 2,410
Operating expenses:				
Research and development	31,642	27,575	95,935	84,778
General and administrative	13,128	11,609	37,628	36,789
Total operating expenses	44,770	39,184	133,563	121,567
Income (loss) from operations	4,444	(38,899)	(49,314)	(119,157)
Other income (expense), net:				
Dividend income and interest income, net	1,960	596	6,084	746
Other income (expense), net	171	(701)	1,296	297
Total other income (expense), net	2,131	(105)	7,380	1,043
Income (loss) before income taxes	6,575	(39,004)	(41,934)	(118,114)
Income tax benefit (provision)	677	—	677	—
Net income (loss)	$ 7,252	$ (39,004)	$ (41,257)	$ (118,114)
Less: net income attributable to participating securities	$ (257)	—	—	—
Net income (loss) attributable to ordinary shareholders, basic and diluted	$ 6,995	$ (39,004)	$ (41,257)	$ (118,114)
Net income (loss) per share attributable to ordinary shareholders—basic	$ 0.07	$ (0.42)	$ (0.39)	$ (1.60)
Weighted-average ordinary shares used in computing net income (loss) per share attributable to ordinary shareholders—basic	106,025,063	93,900,484	104,529,266	73,754,417
Net income (loss) per share attributable to ordinary shareholders—diluted	$ 0.07	$ (0.42)	$ (0.39)	$ (1.60)
Weighted-average ordinary shares used in computing net income (loss) per share attributable to ordinary shareholders—diluted	106,975,231	93,900,484	104,529,266	73,754,417
Other comprehensive income (loss):				
Net income (loss)	$ 7,252	$ (39,004)	$ (41,257)	$ (118,114)
Foreign currency translation	(32)	(76)	(153)	(304)
Comprehensive income (loss)	$ 7,220	$ (39,080)	$ (41,410)	$ (118,418)

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