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

August 3, 2023

Preparing for imminent submission of clinical trial applications (CTAs) for GalNAc-conjugated AATD candidate (WVE-006), the industry's first RNA editing clinical candidate

Novel DMD therapeutic candidate (WVE-N531) with best-in-class exon skipping clinical data is on track to enter a potentially registrational Phase 2 clinical study in 2H 2023, with dystrophin data expected in 2024

"R&D Day" virtual event planned for September 28, 2023; will focus on Wave's leading RNA editing capability and highlight its current and future pipeline of transformative RNA medicines

Cash and cash equivalents of $173.0 million as of June 30, 2023, with runway expected into 2025, plus additional potential milestone payments from GSK collaboration in 2023 and beyond

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

CAMBRIDGE, Mass., Aug. 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 second quarter ended June 30, 2023, and provided a business update.

"In the second quarter, we continued to rapidly advance our pipeline of RNA medicines towards meaningful inflection points. We are on the cusp of moving the industry's first RNA editing therapeutic candidate, WVE-006 for alpha-1 antitrypsin deficiency or AATD, into the clinic, which will mark an important milestone not only for Wave, but also for individuals living with AATD and the entire RNA medicines field. WVE-006 has the potential to transform the standard of care for people living with AATD, and the goal of our initial clinical trial will be delivering early clinical proof-of-concept via measurement of validated serum surrogate markers," said Paul Bolno, MD, MBA, President and Chief Executive Officer of Wave Life Sciences. "While we were disappointed to discontinue WVE-004 for C9-ALS and FTD in the second quarter, we did see successful translation of our preclinical pharmacodynamic effects to clinical biomarker effects, including robust, sustained reductions of up to 50% in poly(GP) with multiple doses. These results are also important validation of our PN chemistry, which is encouraging for our current and upcoming clinical programs, including in DMD, HD, and AATD."

Dr. Bolno continued: "Momentum is building rapidly in our collaboration with GSK, which kicked off at the start of this year, and we are actively working on multiple targets while leveraging proprietary genetic insights through the collaboration. We are excited to share new data on RNA editing disease targets at our annual 'R&D Day' virtual event in September. Wave is well-capitalized to leverage our unique, multimodal platform to develop novel RNA medicines and deliver on a steady cadence of clinical data through 2024 and beyond."

Recent Business Highlights

- **Presented new preclinical data for WVE-006 supporting its potential to be a first-and best-in-class treatment for alpha-1 antitrypsin deficiency (AATD).** In May 2023, at the American Society of Gene & Cell Therapy (ASGCT) Annual Meeting, Wave presented data demonstrating that treatment with WVE-006 improved several markers of liver disease in mice, including a decrease in liver inflammation and hepatocyte turnover and reduction in the size of PAS-D positive globules. WVE-006 is uniquely designed to address both liver and lung disease related to AATD and is poised to be the industry's first RNA editing candidate to reach the clinic. The company expects to submit clinical trial applications (CTAs) for WVE-006 imminently.

- **Highlighted clinical data for WVE-N531 to DMD community at Parent Project Muscular Dystrophy (PPMD) Annual Conference.** In June 2023, in an oral presentation at the PPMD Conference, Wave highlighted its clinical data from the proof-of-concept study of WVE-N531, including the highest level of exon skipping seen in any DMD trial, high muscle concentrations, and a safe and tolerable profile. Wave is on track to initiate dosing in Part B, a potentially registrational Phase 2 trial of WVE-N531, in 2023, which will be powered to evaluate functional dystrophin expression following 24 and 48 weeks of biweekly dosing. 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.

- **Multi-dose portion of SELECT-HD study of WVE-003 for HD underway.** In the second quarter, Wave initiated the multi-dose portion of its ongoing Phase 1b/2a SELECT-HD clinical trial of WVE-003 (allele-selective silencing oligonucleotide) for Huntington's disease, and dosing is currently underway.

- **Emerging best-in-class siRNA designs highlighted at ASGCT.** Also at the May 2023 ASGCT Annual Meeting, Wave highlighted its novel siRNA formats in a poster presentation titled "PN modification of stereopure GalNAc-siRNAs enhances durability of human HSD17B13 silencing in transgenic mouse model." Wave's GalNAc-siRNAs were more potent and had better durability of effect in mice compared with reference GalNAc-siRNA designs, which were driven by improved Ago2

loading. These data were also included in Wave's *Nucleic Acids Research* publication from April 2023, which can be accessed here.

- **Wave Life Sciences added to Russell Indexes.** In June 2023, the company joined the Russell 2000® and Russell 3000® Indexes as part of the annual reconstitution.

Anticipated Upcoming Milestones and Events

WVE-N531 for DMD:

- Initiate dosing in Part B of 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 available multi-dose biomarker and safety clinical data in 2H 2023

Platform and Pipeline:

- "R&D Day" virtual event to be held on September 28, 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

Second Quarter 2023 Financial Results

Wave reported a net loss of $21.1 million in the second quarter of 2023, as compared to $41.3 million in the same period in 2022. The decrease in net loss year-over-year was primarily driven by revenue recognized under the company's collaboration with GSK, which became effective January 27, 2023. Revenue recognized under the GSK and Takeda collaborations in the second quarter of 2023 was $22.1 million. During the second quarter of 2022, revenue of $0.4 million was recognized under the Takeda collaboration.

Research and development expenses were $33.3 million in the second quarter of 2023, as compared to $29.7 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.

General and administrative expenses were $12.3 million in the second quarter of 2023, as compared to $12.8 million in the same period in 2022, primarily due to a decrease in share-based compensation.

As of June 30, 2023, Wave had $173.0 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 second 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)

	June 30, 2023	December 31, 2022
Assets		
Current assets:		
Cash and cash equivalents	$ 172,974	$ 88,497
Prepaid expenses	9,012	7,932
Other current assets	2,722	2,108
Total current assets	184,708	98,537
Long-term assets:		
Property and equipment, net of accumulated depreciation of $40,423 and $37,846 as of June 30, 2023, and December 31, 2022, respectively	14,983	17,284
Operating lease right-of-use assets	24,805	26,843
Restricted cash	3,668	3,660
Other assets	1,821	62
Total long-term assets	45,277	47,849
Total assets	$ 229,985	$ 146,386
Liabilities, Series A preferred shares and shareholders' equity (deficit)		
Current liabilities:		
Accounts payable	$ 12,379	$ 16,915
Accrued expenses and other current liabilities	10,429	17,552
Current portion of deferred revenue	111,133	31,558
Current portion of operating lease liability	6,285	5,496
Total current liabilities	140,226	71,521
Long-term liabilities:		
Deferred revenue, net of current portion	104,540	79,774
Operating lease liability, net of current portion	28,875	32,118
Other liabilities	190	190
Total long-term liabilities	133,605	112,082
Total liabilities	$ 273,831	$ 183,603
Series A preferred shares, no par value; 3,901,348 shares issued and outstanding at June 30, 2023, and December 31, 2022	$ 7,874	$ 7,874
Shareholders' equity (deficit):		
Ordinary shares, no par value; 98,566,816 and 86,924,643 shares issued and outstanding at June 30, 2023, and December 31, 2022, respectively	$ 839,675	$ 802,833
Additional paid-in capital	124,601	119,442
Accumulated other comprehensive income (loss)	(150)	(29)
Accumulated deficit	(1,015,846)	(967,337)
Total shareholders' equity (deficit)	$ (51,720)	$ (45,091)
Total liabilities, Series A preferred shares and shareholders' equity (deficit)	$ 229,985	$ 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 June 30,		Six Months Ended June 30,	
	2023	2022	2023	2022
Revenue	$ 22,106	$ 375	$ 35,035	$ 2,125
Operating expenses:				
Research and development	33,314	29,733	64,293	57,203
General and administrative	12,265	12,806	24,500	25,180
Total operating expenses	45,579	42,539	88,793	82,383

Loss from operations	(23,473)	(42,164)	(53,758)	(80,258)
Other income, net:				
Dividend income and interest income, net	2,251	124	4,124	150
Other income, net	118	744	1,125	998
Total other income, net	2,369	868	5,249	1,148
Loss before income taxes	(21,104)	(41,296)	(48,509)	(79,110)
Income tax provision	—	—	—	—
Net loss	$ (21,104)	$ (41,296)	$ (48,509)	$ (79,110)
Net loss per share attributable to ordinary shareholders—basic and diluted	$ (0.20)	$ (0.62)	$ (0.47)	$ (1.25)
Weighted-average ordinary shares used in computing net loss per share attributable to ordinary shareholders—basic and diluted	105,462,414	66,479,293	103,768,971	63,514,426
Other comprehensive loss:				
Net loss	$ (21,104)	$ (41,296)	$ (48,509)	$ (79,110)
Foreign currency translation	(100)	(142)	(121)	(228)
Comprehensive loss	$ (21,204)	$ (41,438)	$ (48,630)	$ (79,338)

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