Wave Life Sciences Reports First Quarter 2018 Financial Results and Provides Business Update

May 9, 2018

CAMBRIDGE, Mass., May 09, 2018 (GLOBE NEWSWIRE) -- Wave Life Sciences Ltd. (NASDAQ:WVE), a biotechnology company focused on delivering transformational therapies for patients with serious, genetically-defined diseases, today announced financial results for the first quarter ended March 31, 2018, and provided a business update.

“The strong momentum we achieved in 2017 with our clinical development programs and as an organization continued through the first quarter,” said Paul Bolno, MD, MBA, Chief Executive Officer and President of Wave Life Sciences. “We are seeing significant interest from patients and the medical community in our first three clinical studies in Huntington’s disease and Duchenne muscular dystrophy and look forward to initiating three additional development programs by the end of the year.”

First Quarter Highlights and Business Update

- **Ongoing and planned clinical trials on track**

  The PRECISION-HD program, which consists of two global Phase 1b/2a clinical trials evaluating WVE-120101 and WVE-120102 for patients with Huntington’s disease, continues to enroll patients and the company is on track to report topline data in the first half of 2019.

  The global Phase 1 clinical trial, testing WVE-210201 for the treatment of Duchenne muscular dystrophy (DMD) patients amenable to exon 51 skipping, continues to enroll patients. Safety data from the trial are anticipated in the third quarter of 2018.

  The company intends to initiate clinical trials of WVE-3972-01 in amyotrophic lateral sclerosis (ALS) and frontotemporal dementia (FTD) in the fourth quarter of 2018. Wave’s next DMD development program will target exon 53, with clinical trials expected to initiate in the first quarter of 2019.

- **Wave’s manufacturing facility completes inaugural good manufacturing practice (GMP) campaigns**

  Wave is now producing clinical material in the GMP manufacturing suite at its Lexington, Massachusetts facility. The company recently completed manufacturing runs and material from these campaigns will support the development programs, including those for ALS and FTD.

  In July 2017, Wave took occupancy of the new facility which was designed to provide greater independence and flexibility in conducting clinical trials, secure material for current and future development activities and provide the capability for commercial-scale manufacturing. The company is continuing to build out the 90,000-square foot facility with process development, quality control and analytical development laboratories.

- **Takeda collaboration takes effect: at least $230 million in committed cash**

  In April 2018, Wave’s global strategic collaboration with Takeda Pharmaceutical Company Limited to discover, develop and commercialize nucleic acid therapies for disorders of the central nervous system took effect when Wave closed on the issuance and sale of 1,096,892 ordinary shares to Takeda and received aggregate cash proceeds of $60 million. Wave also received an upfront payment of $110 million in cash under the collaboration. In addition, Takeda is required to fund at least $60 million of Wave research over a four-year period to advance multiple preclinical targets.

- **Data presented in April at scientific and medical conferences**

  Wave presented preclinical *in vivo* data at the European Association for the Study of the Liver’s annual meeting, the International Liver Congress 2018, demonstrating that controlling stereochemistry increases both the duration and the potency of GalNAc-conjugated apolipoprotein C-III (APOC3) antisense oligonucleotides, thereby potentially enhancing the pharmacological properties of stereopure oligonucleotides for *APOC3* targeting in the clinic.

  At the 70th Annual Meeting of the American Academy of Neurology, Dr. Robert H. Brown, Jr., Chair and Professor of Neurology at the University of Massachusetts Medical School, presented data from preclinical studies of WVE-3972-01, Wave’s investigational stereopure antisense oligonucleotide designed to target the pathogenic allele of the *C9ORF72* gene for the treatment of ALS and FTD. In preclinical *in vivo* studies, WVE-3972-01 demonstrated a potent, sustained and preferential knockdown of toxic biomarkers associated with ALS and FTD.

- **Neuromuscular research collaboration with Deep Genomics established to identify novel splicing targets**

In April, Wave formed a collaboration with Deep Genomics, Inc. to identify novel therapies to be developed by Wave for the treatment of genetic neuromuscular disorders. Under the collaboration, the companies will analyze and test oligonucleotides against potential therapeutic targets within multiple genes implicated in neuromuscular disorders. The collaboration aims to increase the size of patient populations that may be eligible for treatment by expanding the universe of druggable splicing targets beyond DMD and spinal muscular atrophy.

- **Pfizer nominates maximum number of hepatic targets; collaboration moving toward candidate selection**

  On May 7, 2018, Wave announced that Pfizer recently nominated the fourth and fifth final hepatic targets under the collaboration agreement between the two companies to develop genetically targeted therapies for the treatment of metabolic hepatic diseases, such as nonalcoholic steatohepatitis. Once candidates have been developed, Pfizer may elect to exclusively license the programs and undertake further development and potential commercialization.

First Quarter 2018 Financial Results and Financial Guidance

Wave reported a net loss of $35.2 million in the first quarter of 2018 as compared to $21.1 million in the same period in 2017. The increase in net loss in the first quarter of 2018 was primarily driven by increases in research and development efforts, infrastructure investments and employee headcount to support Wave's corporate goals.

Research and development expenses were $29.2 million in the first quarter of 2018 as compared to $14.7 million in the same period in 2017. The increase in research and development expenses in the first quarter of 2018 was primarily driven by increases in research, preclinical and clinical investments, further expansion of our manufacturing capabilities and facility-related expenses to continue to advance Wave's expanding pipeline.

General and administrative expenses were $8.0 million in the first quarter of 2018 as compared to $5.9 million in the same period in 2017. The increase in general and administrative expenses in the first quarter of 2018 was primarily driven by the continued growth in Wave's employee headcount, as well as increases in facility-related expenses and other general operating expenses.

Wave ended the first quarter of 2018 with $110.5 million in cash and cash equivalents as compared to $142.5 million as of December 31, 2017. The decrease in cash and cash equivalents in the first quarter of 2018 was primarily the result of Wave's quarterly net loss of $35.2 million.

The company expects that its cash and cash equivalents, together with the $170.0 million of cash received from Takeda in April 2018, will enable it to fund its operating and capital expenditure requirements to the end of 2020.

About Wave Life Sciences

Wave Life Sciences is a biotechnology company focused on delivering transformational therapies for patients with serious, genetically-defined diseases. Its chemistry platform enables the creation of highly specific, well characterized oligonucleotides designed to deliver superior efficacy and safety across multiple therapeutic modalities. The company's pipeline is initially focused on neurological disorders and extends across several other therapeutic areas. For more information, please visit www.wavelifesciences.com.

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 commencement, patient enrollment, data readouts and duration of our clinical trials; the protocol, design and endpoints of our clinical trials; the future performance and results of our programs in clinical trials; the progress and potential benefits of our collaborations with partners; the potential of our in vitro and in vivo preclinical data to predict the behavior of our compounds in humans in clinical trials; our identification of future candidates and their therapeutic potential; the anticipated therapeutic benefits of our potential therapies compared to others; our advancing of therapies across multiple modalities and the anticipated benefits of that strategy; the anticipated benefits of our manufacturing process and our internal manufacturing facility; our future growth; the potential benefits of our stereopure compounds compared with stereorandom compounds, our drug discovery platform and nucleic acid therapeutics generally; and the anticipated duration of our cash runway. Actual results may differ materially from those indicated by these forward-looking statements as a result of various important factors, including the following: the ability of our preclinical programs to produce data sufficient to support our clinical trial applications and the timing thereof; our ability to continue to build and maintain the company infrastructure and personnel needed to achieve our goals; the clinical results of our programs, which may not support further development of product candidates; actions of regulatory agencies, which may affect the initiation, timing and progress of clinical trials; our effectiveness in managing future clinical trials and regulatory processes; the success of our platform in identifying viable candidates; the continued development and acceptance of nucleic acid therapeutics as a class of drugs; 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 our collaborators and partners; our ability to manufacture drug material; our ability to obtain, maintain and protect intellectual property; our ability to enforce our patents against infringers and defend our patent portfolio against challenges from third parties; our ability to finance our drug discovery and development efforts and to raise additional capital when needed; and competition from others developing therapies for similar uses, as well as 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, 2018	December 31, 2017
Assets		
Current assets:		
Cash and cash equivalents	$ 110,491	$ 142,503
Prepaid expenses and other current assets	7,470	7,985

Total current assets	117,961	150,488
Long-term assets:		
Property and equipment, net	28,778	27,334
Restricted cash	3,612	3,610
Other assets	70	411
Total long-term assets	32,460	31,355
Total assets	$ 150,421	$ 181,843
Liabilities, Series A preferred shares and shareholders' equity		
Current liabilities:		
Accounts payable	$ 8,014	$ 7,598
Accrued expenses and other current liabilities	6,461	8,898
Current portion of capital lease obligation	—	16
Current portion of deferred rent	70	60
Current portion of deferred revenue	1,275	1,275
Current portion of lease incentive obligation	478	344
Total current liabilities	16,298	18,191
Long-term liabilities:		
Deferred rent, net of current portion	4,591	4,214
Deferred revenue, net of current portion	5,819	7,241
Lease incentive obligation, net of current portion	4,185	3,094
Other liabilities	1,605	1,619
Total long-term liabilities	16,200	16,168
Total liabilities	$ 32,498	$ 34,359
Series A preferred shares, no par value; 3,901,348 shares issued and outstanding at March 31, 2018 and December 31, 2017	$ 7,874	$ 7,874
Shareholders' equity:		
Ordinary shares, no par value; 27,993,337 and 27,829,079 shares issued and outstanding at March 31, 2018 and December 31, 2017, respectively	$ 311,591	$ 310,038
Additional paid-in capital	26,602	22,172
Accumulated other comprehensive income	165	116
Accumulated deficit	(228,309)	(192,716)
Total shareholders' equity	$ 110,049	$ 139,610
Total liabilities, Series A preferred shares and shareholders' equity	$ 150,421	$ 181,843

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,	
	2018	**2017**
Revenue	$ 1,422	$ 383
Operating expenses:		
Research and development	29,196	14,740
General and administrative	8,001	5,850
Total operating expenses	37,197	20,590
Loss from operations	(35,775)	(20,207)
Other income (expense), net:		
Dividend income	356	290
Interest income (expense), net	7	3
Other income (expense), net	343	(72)
Total other income (expense), net	706	221
Loss before income taxes	(35,069)	(19,986)
Income tax provision	(172)	(1,110)
Net loss	$ (35,241)	$ (21,096)
Net loss per share attributable to ordinary shareholders—basic and diluted	$ (1.26)	$ (0.90)

Weighted-average ordinary shares used in computing net loss per share attributable to ordinary shareholders—basic and diluted	27,919,063	23,531,788
Other comprehensive income (loss):		
Foreign currency translation	$ 49	$ 15
Comprehensive loss	$ (35,192)	$ (21,081)

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