Wave Life Sciences Reports Fourth Quarter and Full-Year 2017 Financial Results and Provides Business Update

March 12, 2018

Initiated three clinical trials in 2017 and on track to deliver three additional development programs in 2018

Neurology pipeline growing; candidate in spinocerebellar ataxia type 3 to be named by year end 2018

CAMBRIDGE, Mass., March 12, 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 reported financial results for the fourth quarter and full year ended December 31, 2017, and provided a business update.

"2017 was a transformative year for Wave as we transitioned into clinical development by initiating trials for our three lead neurology programs, established our in-house manufacturing capability and made great progress on delivering three more neurology development programs in 2018," said Paul Bolno, MD, MBA, President and Chief Executive Officer of Wave Life Sciences. "Our expertise in designing potentially first-in-class and innovative medicines continues to grow as we generate additional *in vivo* data demonstrating the impressive pharmacodynamic and pharmacokinetic properties of stereopure oligonucleotides in a variety of animal models across multiple organ systems and tissues. We look forward to advancing our existing and planned clinical programs, collaborating with our partners at Takeda and continuing to build our internal capabilities in preparation for the potential commercialization of our lead programs."

Business Summary and Update

- **Global strategic collaboration with Takeda to advance therapies for central nervous system (CNS) disorders**

  In February 2018, Wave formed a global strategic collaboration with Takeda Pharmaceutical Company Limited (Takeda) to discover, develop, and commercialize nucleic acid therapies for disorders of the CNS. Under the terms of the agreement, Takeda is obligated to make an initial payment of $110 million to Wave and purchase $60 million of Wave's ordinary shares at $54.70 per share. Takeda is also required to fund at least $60 million of Wave research over a four-year period to advance multiple preclinical targets. Wave's collaboration agreement with Takeda will become effective upon satisfaction of customary closing conditions, including the requirements of the Hart-Scott-Rodino Antitrust Improvements Act of 1976.

- **Preclinical *in vivo* data supporting amyotrophic lateral sclerosis (ALS) and frontotemporal dementia (FTD) programs presented at 28th International Symposium on ALS/MND**

  In December 2017, Wave announced data from preclinical studies of WVE-3972-01, the company'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.

- **Neurology pipeline continues to progress and expand across multiple diseases**

  Expanding the neurology pipeline into spinocerebellar ataxia type 3 (SCA3)

  Wave announced today that it expects to name a potential candidate targeting the *ATXN3* gene for the treatment of SCA3 by the end of 2018. This new program will add to Wave's current and planned clinical neurology development programs in Huntington's disease (HD), Duchenne muscular dystrophy (DMD), ALS, and FTD.

  SCA3, also known as Machado–Joseph disease, is caused by a CAG-repeat expansion in the *ATXN3* gene, resulting in an abnormally long polyglutamine stretch in the encoded ataxin-3 protein. Mutant ataxin-3 protein is thought to cause widespread neuronal loss in the brain and spinal cord, likely through a toxic gain of function mechanism. SCA3 is the most common dominantly inherited form of ataxia. The prevalence of SCA3 is believed to be one to two cases in 100,000 people with significant geographic and ethnic variations. There are currently no therapies approved for the treatment of SCA3.

  HD: WVE-120101 and WVE-120102

  The PRECISION-HD program, which includes two global Phase 1b/2a clinical trials evaluating WVE-120101 and WVE-120102 for patients with HD, continues to enroll patients and the company is on track to report topline data in H1 2019.

  Wave's two programs are allele-specific and differentiated from other investigational therapies currently being studied for the treatment of HD. WVE-120101 and WVE-120102 are designed to selectively silence mRNA transcript produced by the disease-causing mutant *huntingtin* (*HTT*) allele. This personalized approach reduces the mutant HTT protein while leaving

the healthy HTT mRNA transcript relatively intact. The healthy transcript is required to produce wild-type, or healthy, HTT protein which is critical for neuronal function, as evidenced by multiple preclinical studies indicating that long-term suppression of healthy HTT protein may have detrimental consequences. Wave's allele-specific approach may also enable the company to address the pre-manifest, or asymptomatic, HD patient population in the future.

DMD: WVE-210201

Wave continues to advance its research and clinical efforts in neuromuscular diseases, including WVE-210201, currently in a global Phase 1 clinical trial for the treatment of DMD patients amenable to exon 51 skipping. Safety data from the trial are anticipated in Q3 2018 and expected to facilitate the rapid transition to an open-label extension study and efficacy study. Both studies following the Phase 1 are designed to include an interim efficacy readout of dystrophin expression from muscle biopsies in H2 2019.

ALS, FTD and exon 53 DMD programs on track to transition to development in 2018

The company intends to initiate clinical trials of WVE-3972-01 in ALS and FTD in Q4 2018. Wave's next DMD development program will target exon 53, with clinical trials expected to initiate in Q1 2019.

- **New *in vivo* data support ophthalmology franchise**

  Wave is advancing the development of stereopure oligonucleotides to target genetic ophthalmologic diseases, with an initial emphasis on retinal diseases. Using the long-noncoding RNA *MALAT1* as a proof-of concept target, a 10-fold increase in potency was achieved *in vivo* with a stereopure oligonucleotide as compared to a stereorandom oligonucleotide following a single intravitreal injection in the back of a mouse eye. The knockdown of *MALAT1* RNA was sustained through three months after the single injection and the study is scheduled to continue for a total of six months.

  In addition, recent results from a preclinical *in vivo* study in non-human primates demonstrated that a stereopure oligonucleotide achieved a clear dose-dependent knockdown of *MALAT1* mRNA in the back of the eye one week following a single intravitreal injection. A six-month duration of effect study is planned.

  Wave is conducting additional research to develop stereopure oligonucleotides against specific genetic targets to treat diseases of the eye.

- **Pfizer collaboration progress**

  In November 2017, Wave achieved a milestone under its collaboration with Pfizer by demonstrating significant activity of stereopure GalNAc-conjugated APOC3 antisense oligonucleotides over stereorandom oligonucleotides in *in vivo* studies and meeting other milestone criteria. The collaboration continues to make progress on developing genetically targeted therapies for the treatment of metabolic diseases, such as nonalcoholic steatohepatitis.

Fourth Quarter and Full Year 2017 Financial Results and Financial Guidance

Wave reported a net loss of $30.2 million in the fourth quarter of 2017 compared to $18.5 million in the fourth quarter of 2016. The company reported a net loss of $102.0 million for the year ended December 31, 2017 as compared to $55.4 million for the year ended December 31, 2016. The increase in net loss for the fourth quarter and year ended December 31, 2017 was mainly due to increases in research and development efforts, infrastructure investments, and employee headcount to support its corporate goals.

Research and development expenses were $25.4 million for the fourth quarter of 2017 as compared to $14.0 million for the same period in 2016. Research and development expenses for the full year were $79.3 million as compared to $40.8 million for the prior year. The increase in research and development expenses for the fourth quarter and full year was primarily driven by increases in research, preclinical and clinical investments, as well as facilities-related expenses to continue to advance Wave's expanding pipeline.

General and administrative expenses were $6.9 million for the fourth quarter of 2017 as compared to $5.2 million for the same period in the prior year. General and administrative expenses were $27.0 million for the full year as compared to $16.0 million for the prior year. The increase in general and administrative expenses in the fourth quarter and full year was primarily driven by the continued growth in Wave's employee headcount, as well as increases in facilities-related expenses and other general operating expenses.

Wave ended 2017 with $142.5 million in cash and cash equivalents compared to $150.3 million as of December 31, 2016. The decrease in cash and cash equivalents was primarily the result of Wave's annual operating loss of $102.0 million partially offset by the $93.5 million in net proceeds from the April 2017 follow-on offering.

The company expects that its cash and cash equivalents, together with the committed cash from its collaboration with Takeda, which is expected to close in the first quarter of 2018, have the potential 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. Our chemistry platform enables the creation of highly specific, well characterized oligonucleotides designed to deliver superior efficacy and safety across multiple therapeutic modalities. Our pipeline is initially focused on neurological disorders and extends across several other therapeutic areas. For more information, please visit www.wavelifesci.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, 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, including the expected timing of when our collaboration with Takeda will take effect; 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 to 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 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 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.

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WAVE LIFE SCIENCES LTD.
UNAUDITED CONSOLIDATED BALANCE SHEETS

(In thousands, except share amounts)

	December 31, 2017	December 31, 2016
Assets		
Current assets:		
Cash and cash equivalents	$ 142,503	$ 150,293
Prepaid expenses and other current assets	7,985	1,483
Deferred tax assets	—	214
Total current assets	150,488	151,990
Long-term assets:		
Property and equipment, net	27,334	8,607
Deferred tax assets	—	560
Restricted cash	3,610	3,601
Other assets	411	53
Total long-term assets	31,355	12,821
Total assets	$ 181,843	$ 164,811
Liabilities, Series A preferred shares and shareholders’ equity		
Current liabilities:		
Accounts payable	$ 7,598	$ 4,943
Accrued expenses and other current liabilities	8,898	4,434
Current portion of capital lease obligation	16	62
Current portion of deferred rent	60	—
Current portion of deferred revenue	2,705	2,705
Current portion of lease incentive obligation	344	11
Total current liabilities	19,621	12,155
Long-term liabilities:		

Capital lease obligation, net of current portion	—	16
Deferred rent, net of current portion	4,214	680
Deferred revenue, net of current portion	5,607	8,311
Lease incentive obligation, net of current portion	3,094	116
Other liabilities	1,619	796
Total long-term liabilities	14,534	9,919
Total liabilities	$ 34,155	$ 22,074
Series A preferred shares, no par value; 3,901,348 shares issued and outstanding	$ 7,874	$ 7,874
Shareholders' equity:		
Ordinary shares, no par value; 27,829,079 and 23,502,169 shares issued and outstanding at December 31, 2017 and 2016, respectively	310,038	215,602
Additional paid-in capital	22,172	10,029
Accumulated other comprehensive income (loss)	116	(291)
Accumulated deficit	(192,512)	(90,477)
Total shareholders' equity	139,814	134,863
Total liabilities, Series A preferred shares and shareholders' equity	$ 181,843	$ 164,811

WAVE LIFE SCIENCES LTD.
UNAUDITED CONSOLIDATED STATEMENTS OF OPERATIONS

(In thousands, except share and per share amounts)

	For the Year Ended December 31,		
	2017	**2016**	**2015**
Revenue	$ 3,704	$ 1,485	$ 152
Operating expenses:			
Research and development	79,309	40,818	9,057
General and administrative	26,975	15,994	10,393
Total operating expenses	106,284	56,812	19,450
Loss from operations	(102,580)	(55,327)	(19,298)
Other income (expense), net:			
Dividend income	1,578	255	—
Interest income (expense), net	6	337	86
Other income (expense), net	(331)	(50)	56
Total other income (expense), net	1,253	542	142
Loss before income taxes	(101,327)	(54,785)	(19,156)
Income tax provision	(708)	(616)	(44)
Net loss	$ (102,035)	$ (55,401)	$ (19,200)
Net loss per share attributable to ordinary shareholders—basic and diluted	$ (3.85)	$ (2.43)	$ (1.83)
Weighted-average ordinary shares used in computing net loss per share attributable to ordinary shareholders—basic and diluted	26,513,382	22,800,628	10,501,455

Source: Wave Life Sciences