Wave Life Sciences Reports Fourth Quarter and Full-Year 2018 Financial Results and Provides Business Update

March 1, 2019

Outlines plans for building a fully integrated genetic medicines company

CAMBRIDGE, Mass., March 01, 2019 (GLOBE NEWSWIRE) -- Wave Life Sciences Ltd. (Nasdaq: WVE), a clinical-stage genetic medicines company committed to delivering life-changing treatments for people battling devastating diseases, today reported financial results for the fourth quarter and full year ended December 31, 2018 and outlined its plans for building a fully integrated company.

"Our achievements throughout 2018 established a strong foundation upon which we are building a world-class and fully integrated genetic medicines company," said Paul Bolno, MD, MBA, President and Chief Executive Officer of Wave Life Sciences. "For 2019, we will remain keenly focused on broadening our pipeline and preparing to launch suvodirsen for the treatment of Duchenne muscular dystrophy in boys who are amenable to exon 51 skipping. Realizing our long-term commitment to those affected by rare genetic diseases will require us to continue to advance our ongoing clinical programs, meaningfully expand into new therapeutic areas such as ophthalmology and continue to invest in and evolve our robust and innovative discovery and drug development platform."

Building a Fully Integrated Genetic Medicines Company

Wave is committed to building upon its discovery, clinical development and manufacturing capabilities and continuing its rapid transformation to become a fully integrated genetic medicines company aspiring to deliver best-in-class medicines. To achieve this ambition, the company is focused on the following priority objectives:

- **Urgently advancing suvodirsen (WVE-210201) toward global commercial launches, including a potential accelerated approval in the United States:** Currently, suvodirsen, the company's investigational therapy for boys with Duchenne muscular dystrophy (DMD) who are amenable to exon 51 skipping, is being studied in an ongoing open-label extension (OLE) study and Wave expects to deliver an interim analysis of dystrophin expression from this study in the second half of 2019. Data from the OLE interim analysis are intended to be an important component of the company's submission to the U.S. Food and Drug Administration (FDA) for accelerated approval in the United States. Also this year, Wave anticipates initiating a global, placebo-controlled Phase 2/3 efficacy and safety clinical trial of suvodirsen. The planned Phase 2/3 trial, which is the first program to be selected for the FDA pilot program for complex innovative trial designs (CID), is designed to measure clinical efficacy and dystrophin expression, and Wave intends to use the results of this trial to seek regulatory approvals globally.

  Wave also intends to make initial investments in commercial capabilities to support the company's transition to a fully integrated, commercial-stage genetic medicines company.

- **Delivering on the PRECISION-HD clinical trials and progressing the pipeline in neuromuscular and central nervous system (CNS) diseases:** The PRECISION-HD program, which consists of two global Phase 1b/2a clinical trials evaluating investigational therapies WVE-120101 and WVE-120102 for patients with Huntington's disease, remains on track to deliver topline data in the first half of 2019. WVE-120101 and WVE-120102, which selectively target the mutant allele of the *huntingtin* (*HTT*) gene, have been shown to reduce levels of mutant *HTT* mRNA and protein, while leaving wild-type or healthy *HTT* mRNA and protein largely intact in *in vitro* studies with patient-derived cell-lines.

  In addition, Wave is developing programs in neuromuscular diseases, including WVE-N531 targeting DMD exon 53 and programs targeting DMD exons 44, 45, 52, 54 and 55, as well as conducting research to identify potential targets for other neuromuscular diseases.

  The company is also advancing WVE-3972-01 in amyotrophic lateral sclerosis and frontotemporal dementia and a lead candidate for spinocerebellar ataxia 3.

  As part of its collaboration with Takeda, Wave is advancing preclinical programs for the treatment of additional CNS diseases, including Alzheimer's disease and Parkinson's disease. Under the terms of the agreement, Wave may collaborate with Takeda on up to six preclinical programs at any one time, during a four-year term. Takeda is funding at least $60 million of Wave's preclinical activities and will reimburse Wave for agreed-upon additional expenses. Takeda is entitled to exclusively license multiple preclinical programs during the term. Wave is eligible for precommercial and commercial milestone payments as well as tiered high single-digit to mid-teen royalty payments on global commercial sales of each licensed program.

- **Selecting first candidate in ophthalmology:** Wave is advancing stereopure oligonucleotides for the potential treatment of inherited retinal diseases. Wave's research in ophthalmology is assessing four inherited retinal diseases, which typically begin in childhood or adolescence and commonly lead to progressive vision loss: retinitis pigmentosa due to a P23H mutation in the RHO gene, Stargardt disease, Usher syndrome type 2A and Leber congenital amaurosis 10. Wave data

presented in October 2018 demonstrated that a single intravitreal injection of stereopure oligonucleotide in the eye of non-human primates resulted in greater than 95% knockdown of a target RNA in the retina for at least four months. Based on these data, the company is working to design clinical candidates that could achieve a therapeutic effect with only two doses per year. The company expects to announce its first ophthalmology candidate in the second half of 2019.

- **Evolving Wave's discovery and drug development platform, PRISM ™:** The company recently branded its proprietary discovery and drug development platform as PRISM. PRISM combines the company's unique ability to construct stereopure oligonucleotides with a deep understanding of how the interplay among oligonucleotide sequence, chemistry and backbone stereochemistry impacts key pharmacological properties. By leveraging artificial intelligence-driven predictive modelling, the company is continuing to explore these interactions to develop an expanding set of design principles that can be applied to a variety of programs across various therapeutic areas.

Fourth Quarter Highlights and Business Update

- **Suvodirsen Phase 2/3 trial chosen for FDA complex innovative trial designs pilot program**

  In January 2019, Wave announced that the planned Phase 2/3 efficacy and safety trial of suvodirsen was selected for the FDA CID pilot program. Through this program, Wave intends to reduce the number of patients required to deliver conclusive clinical efficacy results, thereby minimizing the number of patients required in the placebo treatment arm and potentially accelerating study completion. As a participant in the program, the company will also have additional opportunities to meet with FDA staff to discuss the design elements of the trial, including the use of Bayesian methods to adapt the trial and allow for more efficient and productive clinical determinations.

- **Suvodirsen Phase I results support initiation of Phase 2/3 clinical trial for suvodirsen in DMD**

  In December 2018, Wave announced that the safety and tolerability data from the suvodirsen Phase 1 clinical trial in boys with DMD who are amenable to exon 51 skipping support the initiation of a Phase 2/3 clinical trial. Based on results from the Phase 1 clinical trial and pending final analysis, Wave selected a dose for its planned Phase 2/3 clinical trial of suvodirsen. The company plans to present the results from the Phase 1 clinical trial at upcoming scientific meetings.

- **Hepatic collaboration with Pfizer moving toward candidate selection**

  In 2018, Pfizer completed the selection of targets under the terms of the collaboration agreement between the two companies to develop genetically targeted therapies for the treatment of metabolic hepatic diseases, such as nonalcoholic steatohepatitis. Pfizer has selected five targets, the maximum number of targets permitted under terms of the agreement. Wave is currently advancing programs toward the selection of clinical candidates, at which point Pfizer may elect to exclusively license the programs and undertake further development and potential commercialization.

Fourth Quarter and Full Year 2018 Financial Results and Financial Guidance

Wave reported a net loss of $37.9 million in the fourth quarter of 2018 as compared to $30.8 million in the same period in 2017. The company reported a net loss of $146.7 million for the year ended December 31, 2018 as compared to $102.0 million for the year ended December 31, 2017. The increase in net loss in the fourth quarter and the year ended December 31, 2018 was largely driven by increased research and development efforts and continued organizational growth to support Wave's corporate goals.

Research and development expenses were $39.8 million for the fourth quarter of 2018 as compared to $25.4 million for the same period in 2017. Research and development expenses for the full year were $134.4 million as compared to $79.3 million for the prior year. The increase in research and development expenses in the fourth quarter and full year was primarily due to increases in research, preclinical and clinical activities, further expansion of our manufacturing capabilities and facility-related expenses and related organizational growth to support PRISM.

General and administrative expenses were $12.8 million for the fourth quarter of 2018 as compared to $6.9 million for the same period in the prior year. General and administrative expenses were $39.5 million in 2018 as compared to $27.0 million in 2017. The increase in general and administrative expenses in the fourth quarter and full year was mainly driven by increases in employee headcount to support Wave's corporate goals, as well as increases in professional service expenses and other general operating expenses.

Wave ended 2018 with $174.8 million in cash and cash equivalents as compared to $142.5 million as of December 31, 2017. The increase in cash and cash equivalents was primarily the result of the $170.0 million in cash received from Takeda when the collaboration took effect, which was partially offset by Wave's year-to-date net loss of $146.7 million.

On January 28, 2019, Wave closed a follow-on underwritten public offering of 3,950,000 ordinary shares for gross proceeds of $150.1 million, and on February 26, 2019, Wave closed on the sale of an additional 592,500 ordinary shares pursuant to the underwriters' option (on the same terms and conditions as the initial closing) for gross proceeds of an additional $22.5 million. Net proceeds to Wave from the offering are expected to be approximately $161.6 million, after deducting underwriting discounts and commissions and estimated offering expenses.

Wave expects that its existing cash and cash equivalents, together with expected and committed cash from existing collaborations, will enable Wave to fund its operating and capital expenditure requirements to the end of 2020.

About PRISM ™

PRISM is Wave Life Sciences' proprietary discovery and drug development platform that enables genetically defined diseases to be targeted with stereopure oligonucleotides across multiple therapeutic modalities. PRISM combines the company's unique ability to construct stereopure oligonucleotides with a deep understanding of how the interplay among oligonucleotide sequence, chemistry and backbone stereochemistry impacts key pharmacological properties. By exploring these interactions through iterative analysis of *in vitro* and *in vivo* outcomes and artificial intelligence-driven predictive modeling, the company continues to define design principles that are deployed across programs to rapidly develop and manufacture

clinical candidates that meet pre-defined product profiles.

About Wave Life Sciences

Wave Life Sciences (NASDAQ: WVE) is a clinical-stage genetic 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 commencement, patient enrollment, data readouts and completion of our clinical trials, and the announcement of such events; the protocol, design and endpoints of our ongoing and planned clinical trials; the future performance and results of our programs in clinical trials; future preclinical activities and programs; 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; our identification of future candidates and their therapeutic potential; the anticipated therapeutic benefits of our potential therapies compared to others; our ability to design compounds using multiple modalities and the anticipated benefits of that model; the anticipated benefits of our proprietary manufacturing processes and our internal manufacturing facility; our future growth and anticipated transition to a fully integrated commercial-stage company; the potential benefits of PRISM and our stereopure oligonucleotides compared with stereorandom oligonucleotides; the benefit of nucleic acid therapeutics generally; the strength of our intellectual property; 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: 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; 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 effectiveness of PRISM; the continued development and acceptance of oligonucleotides as a class of medicines; 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 intellectual property; our ability to enforce our patents against infringers and defend our patent portfolio against challenges from third parties; 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)

	December 31, 2018	December 31, 2017
Assets		
Current assets:		
Cash and cash equivalents	$ 174,819	$ 142,503
Current portion of accounts receivable	10,000	1,000
Prepaid expenses and other current assets	17,454	6,985
Total current assets	202,273	150,488
Long-term assets:		
Accounts receivable, net of current portion	50,000	—
Property and equipment, net	39,931	27,334
Restricted cash	3,625	3,610
Other assets	111	411
Total long-term assets	93,667	31,355
Total assets	$ 295,940	$ 181,843
Liabilities, Series A preferred shares and shareholders' equity		
Current liabilities:		
Accounts payable	$ 13,089	$ 7,598
Accrued expenses and other current liabilities	14,736	8,898
Current portion of capital lease obligation	—	16
Current portion of deferred rent	115	60
Current portion of deferred revenue	100,945	1,275
Current portion of lease incentive obligation	1,156	344
Total current liabilities	130,041	18,191
Long-term liabilities:		

Deferred rent, net of current portion	5,132	4,214
Deferred revenue, net of current portion	68,156	7,241
Lease incentive obligation, net of current portion	9,247	3,094
Other liabilities	2,142	1,619
Total long-term liabilities	84,677	16,168
Total liabilities	$ 214,718	$ 34,359
Series A preferred shares, no par value; 3,901,348 shares issued and outstanding at December 31, 2018 and 2017	$ 7,874	$ 7,874
Shareholders' equity:		
Ordinary shares, no par value; 29,472,197 and 27,829,079 shares issued and outstanding at December 31, 2018 and 2017, respectively	375,148	310,038
Additional paid-in capital	37,768	22,172
Accumulated other comprehensive income (loss)	153	116
Accumulated deficit	(339,721)	(192,716)
Total shareholders' equity	73,348	139,610
Total liabilities, Series A preferred shares and shareholders' equity	$ 295,940	$ 181,843

WAVE LIFE SCIENCES LTD.
UNAUDITED CONSOLIDATED STATEMENTS OF OPERATIONS AND COMPREHENSIVE LOSS

(In thousands, except share and per share amounts)

	For the Year Ended December 31,		
	2018	**2017**	**2016**
Revenue	$ 14,414	$ 3,893	$ 1,092
Operating expenses:			
Research and development	134,428	79,309	40,818
General and administrative	39,509	26,975	15,994
Total operating expenses	173,937	106,284	56,812
Loss from operations	(159,523)	(102,391)	(55,720)
Other income (expense), net:			
Dividend income	3,368	1,578	255
Interest income (expense), net	22	6	337
Other income (expense), net	9,549	(331)	(50)
Other income (expense), net	12,939	1,253	542
Loss before income taxes	(146,584)	(101,138)	(55,178)
Income tax benefit (provision)	(69)	(842)	(482)
Net loss	$ (146,653)	$ (101,980)	$ (55,660)
Net loss per share attributable to ordinary shareholders—basic and diluted	$ (5.06)	$ (3.85)	$ (2.44)
Weighted-average ordinary shares used in computing net loss per share attributable to ordinary shareholders—basic and diluted	28,970,404	26,513,382	22,800,628
Other comprehensive income (loss):			
Net loss	$ (146,653)	$ (101,980)	$ (55,660)
Foreign currency translation	37	407	(332)
Comprehensive loss	(146,616)	(101,573)	(55,992)

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