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

November 9, 2018

CAMBRIDGE, Mass., Nov. 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 third quarter ended September 30, 2018 and provided a business update.

“During the third quarter we maintained strong momentum in advancing our three clinical programs and expect to announce our company’s first clinical readout later this year when we share topline safety results from the Phase 1 trial for WVE-210201, our investigational exon 51 skipping candidate for the treatment of Duchenne muscular dystrophy,” said Paul Bolno, MD, MBA, President and Chief Executive Officer of Wave Life Sciences. “Beyond our ongoing clinical programs, we spent much of the last quarter focused on expanding our discovery efforts into inherited retinal diseases and urgently driving additional Duchenne muscular dystrophy programs, including WVE-N531, our investigational exon 53 skipping candidate.”

Third Quarter Highlights and Business Updates

- **WVE-210201 DMD exon 51 skipping program**

  Wave expects to announce topline safety data from its Phase 1 clinical trial evaluating WVE-210201 in Duchenne muscular dystrophy (DMD) patients amenable to exon 51 skipping by the end of the fourth quarter of 2018. As patients complete the Phase 1 trial, they have the option to enroll in an ongoing open-label extension study in which they continue to receive WVE-210201.

  In addition, the company remains on track to deliver an interim analysis of dystrophin expression in muscle biopsies from its ongoing open-label extension study in the second half of 2019.

  Wave has designed a global, pivotal, placebo-controlled Phase 2/3 efficacy and safety clinical trial of WVE-210201 in DMD patients amenable to exon 51 skipping, informed by ongoing discussions with regulatory authorities and the DMD patient community. The study will be powered to assess clinical efficacy and will include dystrophin expression readouts as part of interim and final analyses.

- **WVE-N531 DMD exon 53 skipping program and additional DMD exon skipping programs**

  Wave is leveraging learnings from its ongoing DMD development and discovery efforts to advance WVE-N531, its preclinical program to target DMD in boys amenable to exon 53 skipping. Recent data presented at the 23rd International Annual Congress of the World Muscle Society demonstrated that WVE-N531 induced up to 71% dystrophin protein restoration *in vitro* in DMD patient-derived myoblasts compared with healthy human myoblasts as measured by Western Blot. Subject to submission of clinical trial applications and approval to proceed, Wave expects to deliver topline clinical data for WVE-N531 in the second half of 2020.

  The company is also expanding its DMD discovery programs to explore additional exon targets beyond its current exon 51 and exon 53 skipping programs, specifically including exons 44, 45, 52, 54 and 55.

- **Pipeline expansion to rare, genetic eye diseases**

  Last month, Wave announced plans to design and advance stereopure oligonucleotide therapeutics 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’s decision to expand its therapeutic pipeline into ophthalmology is supported by its data presented at the 14th Annual Meeting of the Oligonucleotide Therapeutics Society in October 2018. The data demonstrate 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 development candidates that could achieve a therapeutic effect with only two doses per year.

  Wave expects to announce its first ophthalmology development candidate in the second half of 2019.

- **PRECISION-HD Phase 1b/2a clinical trials**

  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. An open-label extension study to assess safety, tolerability and efficacy using validated clinical outcome measures is planned for patients as they complete the ongoing Phase 1b/2a trials.

- **Continued validation of Wave's stereochemistry platform**

  Wave shared advancements related to its novel stereochemistry platform at the 14th Annual Meeting of the Oligonucleotide Therapeutics Society in October 2018. The company's latest findings provide further validation of Wave's platform to precisely design, optimize and manufacture stereopure oligonucleotides. Presentations included preclinical data demonstrating that Wave's stereochemical control of antisense oligonucleotides enhances target efficacy and enables broad tissue distribution and exposure.

Third Quarter 2018 Financial Results and Financial Guidance

Wave reported a net loss of $37.6 million in the third quarter of 2018 as compared to $25.5 million in the same period in 2017. The increase in net loss in the third quarter of 2018 was largely driven by increased research and development efforts to support achievement of Wave's corporate goals.

Research and development expenses were $32.9 million in the third quarter of 2018 as compared to $20.1 million in the same period in 2017. The increase in research and development expenses in the third quarter of 2018 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 Wave's advancing and expanding pipeline.

General and administrative expenses were $9.8 million in the third quarter of 2018 as compared to $7.6 million in the same period in 2017. The increase in general and administrative expenses in the third quarter of 2018 was mainly driven by the increase in employee headcount, as well as increases in professional services and other general operating expenses.

Wave ended the third quarter of 2018 with $210.5 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 of cash received from Takeda, which was partially offset by Wave's year-to-date net loss of $108.8 million.

Wave expects that its existing cash and cash equivalents, together with expected and committed cash from existing collaborations, 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 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 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 model; 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; 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 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 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)

	September 30, 2018	December 31, 2017
Assets		
Current assets:		

Cash and cash equivalents	$ 210,489	$ 142,503
Current portion of accounts receivable	10,000	1,000
Prepaid expenses and other current assets	12,672	6,985
Total current assets	233,161	150,488
Long-term assets:		
Accounts receivable, net of current portion	50,000	—
Property and equipment, net	37,722	27,334
Restricted cash	3,620	3,610
Other assets	74	411
Total long-term assets	91,416	31,355
Total assets	$ 324,577	$ 181,843
Liabilities, Series A preferred shares and shareholders' equity		
Current liabilities:		
Accounts payable	$ 11,961	$ 7,598
Accrued expenses and other current liabilities	9,518	8,898
Current portion of capital lease obligation	—	16
Current portion of deferred rent	90	60
Current portion of deferred revenue	103,229	1,275
Current portion of lease incentive obligation	997	344
Total current liabilities	125,795	18,191
Long-term liabilities:		
Deferred rent, net of current portion	5,084	4,214
Deferred revenue, net of current portion	69,494	7,241
Lease incentive obligation, net of current portion	8,229	3,094
Other liabilities	1,495	1,619
Total long-term liabilities	84,302	16,168
Total liabilities	$ 210,097	$ 34,359
Series A preferred shares, no par value; 3,901,348 shares issued and outstanding at September 30, 2018 and December 31, 2017	$ 7,874	$ 7,874
Shareholders' equity:		
Ordinary shares, no par value; 29,426,176 and 27,829,079 shares issued and outstanding at September 30, 2018 and December 31, 2017, respectively	$ 374,502	$ 310,038
Additional paid-in capital	33,757	22,172
Accumulated other comprehensive income	181	116
Accumulated deficit	(301,834)	(192,716)
Total shareholders' equity	$ 106,606	$ 139,610
Total liabilities, Series A preferred shares and shareholders' equity	$ 324,577	$ 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 September 30,		Nine Months Ended September 30,	
	2018	2017	2018	2017
Revenue	$ 4,493	$ 1,315	$ 10,794	$ 2,795
Operating expenses:				
Research and development	32,876	20,097	94,619	53,940
General and administrative	9,849	7,571	26,755	20,088
Total operating expenses	42,725	27,668	121,374	74,028
Loss from operations	(38,232)	(26,353)	(110,580)	(71,233)
Other income (expense), net:				
Dividend income	1,064	515	2,354	1,287
Interest income (expense), net	5	1	16	5
Other income (expense), net	(468)	(75)	(384)	(211)

Total other income (expense), net	601	441	1,986	1,081
Loss before income taxes	(37,631)	(25,912)	(108,594)	(70,152)
Income tax benefit (provision)	—	418	(172)	(1,035)
Net loss	$ (37,631)	$ (25,494)	$ (108,766)	$ (71,187)
Net loss per share attributable to ordinary shareholders—basic and diluted	$ (1.28)	$ (0.92)	$ (3.78)	$ (2.73)
Weighted-average ordinary shares used in computing net loss per share attributable to ordinary shareholders—basic and diluted	29,333,994	27,758,792	28,804,357	26,078,696
Other comprehensive income (loss):				
Net loss	$ (37,631)	$ (25,494)	$ (108,766)	$ (71,187)
Foreign currency translation	(20)	1	65	19
Comprehensive loss	$ (37,651)	$ (25,493)	$ (108,701)	$ (71,168)

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