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

August 9, 2018

CAMBRIDGE, Mass. , Aug. 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 second quarter ended June 30, 2018 and provided a business update.

“We continue to advance our programs for the treatment of Duchenne muscular dystrophy with preparations underway for our global, pivotal trial of investigational WVE-210201 for boys amenable to exon 51 skipping. Recently presented preclinical data demonstrating that our stereopure oligonucleotides can restore up to 90% of natural dystrophin in an animal model underscore the significant potential of our platform to address Duchenne muscular dystrophy,” said Paul Bolno, MD, MBA, President and Chief Executive Officer of Wave Life Sciences . “Through the rest of the year, we look forward to sharing Phase 1 safety data on WVE-210201, presenting advances in our Duchenne muscular dystrophy exon 53 skipping program, and maintaining strong momentum in the ongoing PRECISON-HD clinical program, the first clinical study conducted in the United States using an oligonucleotide approach for Huntington’s disease.”

Second Quarter Highlights and Business Update

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

  The ongoing single ascending dose Phase 1 clinical trial of WVE-210201 in Duchenne muscular dystrophy (DMD) patients amenable to exon 51 skipping continues to progress through the planned dose escalation with no safety signals observed in the trial. Wave now expects to announce safety data from the Phase 1 trial by the end of the fourth quarter of 2018. Patient interest and enrollment in the trial continue to be strong. As patients complete the Phase 1 trial, they have the option to enroll in an ongoing open label extension study in which they receive continued treatment with WVE-210201.

  Wave remains on track to deliver an interim efficacy readout of dystrophin expression from muscle biopsies from ongoing and planned clinical trials in the second half of 2019.

  Wave has designed a global, pivotal, placebo-controlled Phase 2/3 efficacy and safety study of WVE-210201 in DMD patients amenable to exon 51 skipping, informed by ongoing discussions with global 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.

  In addition, the positive opinion by the European Medicines Agency Committee for Orphan Medical Products recommending WVE-210201 for designation as an orphan medicinal product for the treatment of DMD was adopted by the European Commission .

- **DMD exon 53 targeting program**

  Wave is leveraging learnings from its ongoing DMD development and discovery efforts to advance its program to address DMD in boys amenable to exon 53 skipping, including recent data presented at the Project Parent Muscular Dystrophy (PPMD) Annual Conference. The company will present new data from its exon 53 skipping program at the 23rd International Annual Congress of the World Muscle Society in October 2018 and expects to deliver a clinical data readout for this program in 2020.

- **PRECSION-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, continues to enroll patients at sites in the United States , Europe and Canada , and the company is on track to report topline data in the first half of 2019.

- **Promising *in vivo* data presented for ongoing and planned programs in DMD, ALS and FTD**

  At the PPMD Annual Conference on June 29, 2018 , the company presented *in vivo* data demonstrating that its murine-specific stereopure oligonucleotide restored 70% to 90% of natural dystrophin in a dystrophin deficient mouse model for DMD (*mdx*23) with substantial protein expression in the heart and diaphragm. Wave also provided an update on its clinical and discovery programs in DMD at the 2018 New Directions in Biology and Disease of Skeletal Muscle Conference .

  In the last three months, Wave has presented *in vivo* preclinical study results for WVE-3972-01, the company’s investigational stereopure antisense oligonucleotide designed to target the pathogenic allele of the *C9ORF72* gene for the treatment of amyotrophic lateral sclerosis (ALS) and frontotemporal dementia (FTD). Animal data for WVE-3972-01

demonstrating potent, sustained and preferential knockdown of toxic biomarkers associated with ALS and FTD were presented at the Alzheimer's Association International Conference , RNA Therapeutics Conference 2018: From Base Pairs to Bedside and the 5th International Conference on Molecular Neurodegeneration . The company intends to initiate clinical trials of WVE-3972-01 in ALS and FTD in the fourth quarter of 2018.

- **Continued strengthening of intellectual property position**

  Wave's advances with its core chemistry and stereochemistry platform continue to strengthen its intellectual property position relating to the design, synthesis and manufacture of stereopure nucleic acid therapeutic candidates, including the allowance of sequence-independent composition-of-matter claims in the United States.

Second Quarter 2018 Financial Results and Financial Guidance

Wave reported a net loss of $35 .9 million in the second quarter of 2018 as compared to $24 .6 million in the same period in 2017. The increase in net loss in the second quarter of 2018 was largely driven by increased research and development efforts and the continued growth of employee headcount to support Wave's programs.

Research and development expenses were $32 .5 million in the second quarter of 2018 as compared to $19 .1 million in the same period in 2017. The increase in research and development expenses in the second quarter of 2018 was largely driven by increases in research, preclinical and clinical investments, further expansion of our manufacturing capabilities and facility-related expenses, along with the continued growth of employee headcount to support Wave's programs.

General and administrative expenses were $8 .9 million in the second quarter of 2018 as compared to $6 .7 million in the same period in 2017. The increase in general and administrative expenses in the second quarter of 2018 was mainly driven by the increase in Wave's employee headcount, as well as increases in professional service expenses and other general operating expenses.

Wave ended the second quarter of 2018 with $241 .4 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 net loss of $71 .1 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; the protocol, design and endpoints of our ongoing and planned 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 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)

	June 30, 2018	December 31, 2017
Assets		
Current assets:		
Cash and cash equivalents	$ 241,431	$ 142,503
Current portion of accounts receivable	15,000	1,000

Prepaid expenses and other current assets	11,243	6,985
Total current assets	267,674	150,488
Long-term assets:		
Accounts receivable, net of current portion	50,000	—
Property and equipment, net	32,384	27,334
Restricted cash	3,616	3,610
Other assets	69	411
Total long-term assets	86,069	31,355
Total assets	$ 353,743	$ 181,843
Liabilities, Series A preferred shares and shareholders' equity		
Current liabilities:		
Accounts payable	$ 7,151	$ 7,598
Accrued expenses and other current liabilities	8,494	8,898
Current portion of capital lease obligation	—	16
Current portion of deferred rent	80	60
Current portion of deferred revenue	27,294	1,275
Current portion of lease incentive obligation	762	344
Total current liabilities	43,781	18,191
Long-term liabilities:		
Deferred rent, net of current portion	4,864	4,214
Deferred revenue, net of current portion	149,921	7,241
Lease incentive obligation, net of current portion	6,474	3,094
Other liabilities	1,533	1,619
Total long-term liabilities	162,792	16,168
Total liabilities	$ 206,573	$ 34,359
Series A preferred shares, no par value; 3,901,348 shares issued and outstanding at June 30, 2018 and December 31, 2017	$ 7,874	$ 7,874
Shareholders' equity:		
Ordinary shares, no par value; 29,293,350 and 27,829,079 shares issued and outstanding at June 30, 2018 and December 31, 2017, respectively	$ 373,151	$ 310,038
Additional paid-in capital	30,147	22,172
Accumulated other comprehensive income	201	116
Accumulated deficit	(264,203)	(192,716)
Total shareholders' equity	$ 139,296	$ 139,610
Total liabilities, Series A preferred shares and shareholders' equity	$ 353,743	$ 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 June 30,		Six Months Ended June 30,	
	2018	2017	2018	2017
Revenue	$ 4,879	$ 1,097	$ 6,301	$ 1,480
Operating expenses:				
Research and development	32,547	19,103	61,743	33,843
General and administrative	8,905	6,667	16,906	12,517
Total operating expenses	41,452	25,770	78,649	46,360
Loss from operations	(36,573)	(24,673)	(72,348)	(44,880)
Other income (expense), net:				
Dividend income	934	482	1,290	772
Interest income (expense), net	4	1	11	4
Other income (expense), net	(259)	(64)	84	(136)
Total other income (expense), net	679	419	1,385	640
Loss before income taxes	(35,894)	(24,254)	(70,963)	(44,240)

Income tax provision	—	(343)	(172)	(1,453)
Net loss	$ (35,894)	$ (24,597)	$ (71,135)	$ (45,693)
Net loss per share attributable to ordinary shareholders—basic and diluted	$ (1.23)	$ (0.91)	$ (2.49)	$ (1.81)
Weighted-average ordinary shares used in computing net loss per share attributable to ordinary shareholders—basic and diluted	29,144,466	26,899,058	28,535,149	25,224,725
Other comprehensive income (loss):				
Foreign currency translation	$ 36	$ 3	$ 85	$ 18
Comprehensive loss	$ (35,858)	$ (24,594)	$ (71,050)	$ (45,675)

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