



## Wave Life Sciences Reports Third Quarter 2017 Financial Results and Provides Business Update

November 9, 2017

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Nov. 9, 2017-- 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, 2017.

"Through the growth of talent and capabilities in our organization and excellent execution throughout the quarter, we achieved our ambitious clinical and research goals. Importantly, we initiated our third clinical program of 2017 with a Phase 1 study investigating our DMD Exon 51 program, WVE-210201," said Paul Bolno, MD, MBA, President and Chief Executive Officer of Wave Life Sciences. "Our first peer reviewed publication was featured in *Nature Biotechnology*, highlighting the founding principles of Wave's stereopure chemistry platform and approach. With the initiation of our first three clinical programs this year and another three programs recently announced, we are on track to meet our objective of six development programs underway by the end of 2018."

### Business Update

- **DMD Exon 51 (WVE-210201) program initiates clinical trial**

On November 6, Wave announced the initiation of clinical development for WVE-210201, Wave's lead program for Duchenne muscular dystrophy (DMD) patients amenable to exon 51 skipping. We expect that data from the Phase 1 trial for WVE-210201 will be available in Q3 2018 and will 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 2H 2019. The company is exploring both intravenous and subcutaneous administration.

- **Huntington's disease allele selective programs (WVE-120101, WVE-120102) clinical trials on track**

The company's PRECISION-HD program, which includes two Phase 1b/2a global clinical trials evaluating WVE-120101 and WVE-120102 for patients with Huntington's disease (HD), continues to enroll and it is on track to report topline data in 1H 2019.

- **First peer-reviewed publication in *Nature Biotechnology***

In August, Wave announced its first peer-reviewed publication in the September issue of *Nature Biotechnology*, representing a significant scientific advancement for the oligonucleotide field. The paper, entitled "Control of phosphorothioate stereochemistry substantially increases the efficacy of antisense oligonucleotides," describes a breakthrough method to produce antisense oligonucleotide (ASO) therapeutics with high stereochemical purity as well as rational drug design to control pharmacologic properties in nucleic acid therapeutics drug development more broadly.

The paper outlines early foundational principles discovered by Wave to engage RNase H1 that can be applied to any ASO sequence, and demonstrated that stereochemistry plays a central role in oligonucleotide drug design, with the potential to improve stability, duration of activity and specificity. The company's highly efficient manufacturing process may allow for these key findings to translate into breakthrough treatments by applying nucleic acid therapeutics. Wave is continuing to leverage these initial findings to further build its knowledge base and expand its platform capabilities beyond antisense, including ongoing work in exon skipping, single stranded RNAi and other modalities.

- **Pfizer collaboration progress**

The collaboration with Pfizer continues to make progress on developing genetically targeted therapies for the treatment of metabolic diseases, including nonalcoholic steatohepatitis (NASH). As an update, our APOC3-GalNAc ASO findings demonstrated a potency equivalent to state-of-the-art GalNAc conjugated double-strand RNAi with a median effective dose of 0.3mg/kg *in vivo*. In addition to a 7- to 10-fold improvement in potency, an increase was seen in durability over GalNAc conjugated stereorandom APOC3.

The collaboration has accelerated the application of Wave's stereochemistry platform across antisense and RNAi modalities with the incorporation of GalNAc and Pfizer's hepatic targeting technology to address disease of the liver. Wave continues to advance its research efforts in this area.

In addition, Wave amended its collaboration agreement with Pfizer to extend the target nomination period from November 5, 2017 to May 5, 2018, which provides Pfizer with an additional six months to nominate the two remaining hepatic targets under the agreement.

Wave reported a net loss of \$26.1 million for the third quarter of 2017, as compared to a net loss of \$17.5 million for the third quarter of 2016. The increase in net loss for the third quarter was mainly due to increases in research and development supplies and services expenses and increases in compensation-related expenses as Wave continues to grow its employee headcount to support its corporate goals.

Research and development expenses were \$20.1 million for the third quarter of 2017 as compared to \$13.7 million for the third quarter of 2016. The increase for the third quarter was driven by increases in research, discovery, development, preclinical and clinical supplies and services expenses, as well as increases in compensation-related expenses, related to the continued growth in employee headcount, and increases in facility-related expenses.

General and administrative expenses were \$7.6 million for third quarter of 2017, as compared to \$3.9 million for the third quarter of 2016. This increase for the third quarter was due to increases in compensation-related expenses, related to the continued growth in Wave's employee headcount, as well as increases in facility-related expenses and other general operating expenses.

As of September 30, 2017, Wave had cash and cash equivalents totaling \$168.5 million as compared to \$150.3 million as of December 31, 2016. The increase in cash and cash equivalents was due to the \$93.5 million in net proceeds from the April 2017 follow-on offering which was partially offset by Wave's year-to-date net loss of \$71.8 million.

Wave expects that the capital resources available as of September 30, 2017, together with anticipated milestone payments under its existing collaboration with Pfizer, will be sufficient to fund its operating expenses and capital expenditure requirements into mid-2019.

## 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.

## Forward-Looking Information

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 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; 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 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.

## WAVE LIFE SCIENCES LTD. UNAUDITED CONSOLIDATED BALANCE SHEETS

*(In thousands, except share amounts)*

	<u>September 30, 2017</u>	<u>December 31, 2016</u>
<b>Assets</b>		
Current assets:		
Cash and cash equivalents	\$ 168,464	\$ 150,293
Prepaid expenses and other current assets	5,370	1,483
Deferred tax assets	—	214
Total current assets	<u>173,834</u>	<u>151,990</u>
Property and equipment, net	24,584	8,607
Deferred tax assets	—	560
Restricted cash	3,608	3,601
Other assets	60	53
Total assets	<u>\$ 202,086</u>	<u>\$ 164,811</u>
<b>Liabilities, Series A preferred shares and shareholders' equity</b>		
Current liabilities:		
Accounts payable	\$ 4,705	\$ 4,943
Accrued expenses and other current liabilities	6,953	4,434
Current portion of capital lease obligation	31	62
Current portion of deferred rent	50	—
Current portion of deferred revenue	2,705	2,705

Current portion of lease incentive obligation	678	11
Total current liabilities	<u>15,122</u>	<u>12,155</u>
Long-term liabilities:		
Capital lease obligation, net of current portion	—	16
Deferred rent, net of current portion	3,837	680
Deferred revenue, net of current portion	6,283	8,311
Lease incentive obligation, net of current portion	2,093	116
Other liabilities	<u>1,021</u>	<u>796</u>
Total long-term liabilities	<u>13,234</u>	<u>9,919</u>
Total liabilities	<u>\$ 28,356</u>	<u>\$ 22,074</u>
Series A preferred shares, no par value; 3,901,348 shares issued and outstanding at September 30, 2017 and December 31, 2016	<u>\$ 7,874</u>	<u>\$ 7,874</u>
Shareholders' equity:		
Ordinary shares, no par value; 27,767,905 and 23,502,169 shares issued and outstanding at September 30, 2017 and December 31, 2016, respectively	\$ 309,434	\$ 215,602
Additional paid-in capital	18,995	10,029
Accumulated other comprehensive loss	(272)	(291)
Accumulated deficit	<u>(162,301)</u>	<u>(90,477)</u>
Total shareholders' equity	<u>\$ 165,856</u>	<u>\$ 134,863</u>
Total liabilities, Series A preferred shares and shareholders' equity	<u>\$ 202,086</u>	<u>\$ 164,811</u>

**WAVE LIFE SCIENCES LTD.**  
**UNAUDITED CONSOLIDATED STATEMENTS OF OPERATIONS**

*(In thousands, except share and per share amounts)*

	<b>Three Months Ended September 30,</b>		<b>Nine Months Ended September 30,</b>	
	<b>2017</b>	<b>2016</b>	<b>2017</b>	<b>2016</b>
Revenue	\$ 676	\$ 392	\$ 2,028	\$ 809
Operating expenses:				
Research and development	20,097	13,686	53,940	26,823
General and administrative	7,571	3,939	20,088	10,809
Total operating expenses	<u>27,668</u>	<u>17,625</u>	<u>74,028</u>	<u>37,632</u>
Loss from operations	(26,992)	(17,233)	(72,000)	(36,823)
Other income (expense):				
Dividend income	515	—	1,287	—
Interest income (expense), net	1	118	5	328
Other income (expense), net	(75)	(36)	(211)	(25)
Total other income (expense), net	<u>441</u>	<u>82</u>	<u>1,081</u>	<u>303</u>
Loss before income taxes	(26,551)	(17,151)	(70,919)	(36,520)
Income tax benefit (provision)	416	(384)	(905)	(427)
Net loss	<u>\$ (26,135)</u>	<u>\$ (17,535)</u>	<u>\$ (71,824)</u>	<u>\$ (36,947)</u>
Net loss per share attributable to ordinary shareholders— basic and diluted	<u>\$ (0.94)</u>	<u>\$ (0.75)</u>	<u>\$ (2.75)</u>	<u>\$ (1.64)</u>
Weighted-average ordinary shares used in computing net loss per share attributable to ordinary shareholders—basic and diluted	<u>27,758,792</u>	<u>23,445,673</u>	<u>26,078,696</u>	<u>22,571,575</u>

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