



## WAVE Life Sciences Reports First Quarter 2016 Financial Results and Provides Business Update

May 16, 2016

*On track to deliver six clinical programs by 2018, including two IND filings in 2016*

*Entered collaboration with Pfizer to develop genetically targeted therapies for the treatment of metabolic diseases*

*Existing cash supports pipeline progression, clinical programs and operations into 2019*

CAMBRIDGE, Mass.--(BUSINESS WIRE)--May 16, 2016-- WAVE Life Sciences Ltd. (NASDAQ: WVE), a genetic medicines company focused on developing stereopure nucleic acid therapies for patients impacted by rare diseases, today reported financial results for the first quarter ended March 31, 2016 and provided a business update.

"Building on our tremendous momentum from 2015, we have achieved a series of key milestones that have positioned us for continued growth in 2016 and beyond," said Paul Bolno, M.D., MBA, President and Chief Executive Officer of WAVE Life Sciences. "In January, we announced a clear strategic plan for our future, focused on developing a portfolio of medicines for patients with neurological and neuromuscular diseases while leveraging the versatility of our platform to work alongside partners who have deep expertise in other therapeutic areas. In this first quarter, we commenced IND-enabling studies for our HD HTT SNP-1 program and selected lead candidates for our HD HTT SNP-2 program and our exon 51 DMD program. In addition, we entered into a collaboration with Pfizer validating our externalization strategy."

### Pipeline Update and Outlook

- **Huntington's Disease (HD) Programs.** WAVE is on track to file two investigational new drug (IND) applications in late 2016 for its two lead allele-specific antisense programs and to commence its first HD clinical trial in 2017. Each program distinctly targets the underlying genetic cause of HD and between these two programs, a substantial majority of HD patients would be covered. WAVE has established strong capabilities in the manufacturing and scale-up process for its stereopure nucleic acid therapies, which have produced materials for its IND-enabling studies and will continue to meet its needs to enter the clinic. WAVE continues to build strong relationships with the Huntington's Disease patient community.
- **Duchenne Muscular Dystrophy (DMD) Programs.** WAVE expects to file an IND and commence the first clinical trial for its exon 51 DMD candidate in 2017. The DMD clinical trials will be rigorously designed and include ambulatory and non-ambulatory patients. WAVE's preclinical data demonstrate 25-fold enhanced exon-skipping efficiency (in vitro) and broader distribution (in DMD animal models), including to heart and diaphragm, compared to other exon-skipping therapies under investigation. These and additional data will be presented at the upcoming Parent Project Muscular Dystrophy (PPMD) Annual Connect Conference in June 2016. In addition, WAVE extended its research collaboration with the University of Oxford and renowned researcher Matthew Wood to accelerate additional exon-skipping programs beyond exon 51.
- **Pfizer Collaboration.** On May 5, 2016, WAVE entered into a collaboration with Pfizer. This collaboration will focus on genetically defined targets for the treatment of metabolic diseases and bring together WAVE's proprietary stereopure drug development platform, across antisense and RNAi modalities, along with GalNAc and Pfizer's hepatic targeting technology for enhanced delivery to the liver. WAVE has agreed to advance up to five programs from discovery through to the selection of clinical candidates, at which point Pfizer may elect to exclusively license the programs. Under the collaboration, Pfizer agreed to pay WAVE \$40 million in upfront cash (of which \$30 million was in exchange for WAVE equity) and up to an additional \$871 million in potential research, development and commercial milestone payments, plus royalties, tiered up to low double-digits, on sales of any products that may result from the collaboration.
- **Liver Programs.** In addition to its collaboration with Pfizer in liver, WAVE is using its innovative and proprietary synthetic chemistry drug development platform to develop stereopure antisense and RNAi therapeutics for liver diseases. The company expects to report data from its GalNAc proof of concept, subcutaneously administered, non-human primate studies in the second half of 2016.

### First Quarter 2016 Financial Results and Financial Guidance

WAVE reported a net loss of \$7.8 million in the first quarter of 2016 as compared to a net loss of \$3.5 million in the first quarter of 2015. The increase in net loss for the first quarter was largely due to increased research and development efforts as well as investments in the company's infrastructure as a publicly traded company.

Research and development expenses were \$4.7 million for the first quarter of 2016 as compared to \$1.6 million for the same period in 2015. The increase in research and development expenses in the quarter was primarily driven by increased spending on third party-related costs used to advance WAVE's preclinical and drug discovery activities, as well as increased salary and related benefits costs due to the increase in employee headcount.

General and administrative expenses were \$3.2 million for the first quarter of 2016 as compared to \$1.9 million for the same period in the prior year. The increase in general and administrative expenses in the first quarter was primarily due to an increase in professional fees and outside support services due to the costs of being a public company, as well as salary and benefits increases due to an increase in headcount.

WAVE's cash as of March 31, 2016 was \$153.4 million compared to \$161.2 million as of December 31, 2015. The decrease in cash was primarily the result of our net loss for the first quarter of 2016.

WAVE expects that the cash resources it had on hand at March 31, 2016, together with the \$40.0 million in upfront cash payments under the May 2016 Pfizer collaboration, will fund its operating expenses and capital expenditure requirements into 2019.

#### About WAVE Life Sciences

At WAVE Life Sciences, we are driven by an unwavering passion and commitment to deliver on our mission of confronting challenging diseases by developing transformational therapies and empowering patients. We are utilizing our innovative and proprietary synthetic chemistry drug development platform to design, develop and commercialize stereopure nucleic acid therapeutics that precisely target the underlying cause of rare and other serious genetically defined diseases. Given the versatility of our chemistry platform, WAVE's deep, diverse [pipeline](#) spans multiple modalities including antisense, exon-skipping, and single-stranded RNAi. For more information, please visit [www.wavelifesciences.com](http://www.wavelifesciences.com) and follow us on Twitter at [@WAVELifeSci](#) and [LinkedIn](#).

#### 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 following: our filing of INDs and commencing clinical trials; the future performance and results of our programs in clinical trials; our identification of future candidates and their therapeutic potential; the anticipated therapeutic benefits of stereopure therapies compared to other therapies; our advancing of therapies across multiple modalities and the anticipated benefits of that strategy; our future growth; the potential of our stereopure approach and nucleic acid therapeutics; and our cash position supporting our anticipated business activities. 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 the filing of INDs 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 stereopure 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 raise additional capital as needed; and competition from others developing therapies for similar uses, as well as the information under the caption "Risk Factors" contained in our Annual Report on Form 10-K filed with the Securities and Exchange Commission (SEC) on March 30, 2016 and in other filings we make with the SEC. 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 CONDENSED CONSOLIDATED BALANCE SHEETS

*(In thousands, except share amounts)*

	<u>March 31, 2016</u>	<u>December 31, 2015</u>
<b>Assets</b>		
Current Assets:		
Cash	\$ 153,397	\$ 161,220
Prepaid expenses and other current assets	580	146
Deferred tax assets	23	18
Total current assets	<u>154,000</u>	<u>161,384</u>
Property and equipment, net	3,218	2,789
Deferred tax assets	192	192
Restricted cash	1,055	1,055
Other assets	57	4
Total assets	<u>\$ 158,522</u>	<u>\$ 165,424</u>
<b>Liabilities, Series A preferred shares and shareholders' equity</b>		
Current Liabilities:		
Accounts payable	\$ 2,437	\$ 2,811
Accrued expenses and other current liabilities	1,271	945
Current portion of capital lease obligation	62	62
Total current liabilities	<u>3,770</u>	<u>3,818</u>
Long-term liabilities:		
Capital lease obligation, net of current portion	62	78
Other liabilities	295	163
Total long-term liabilities	<u>357</u>	<u>241</u>
Total liabilities	<u>\$ 4,127</u>	<u>\$ 4,059</u>
Series A preferred shares, no par value; 3,901,348 shares issued and outstanding	<u>7,874</u>	<u>7,874</u>
Shareholders' equity:		
Ordinary shares, no par value; 21,551,423 shares issued and outstanding	185,344	185,344
Additional paid-in capital	4,048	3,182
Accumulated other comprehensive income	52	41
Accumulated deficit	<u>(42,923)</u>	<u>(35,076)</u>
Total shareholders' equity	<u>146,521</u>	<u>153,491</u>
Total liabilities, Series A preferred shares and shareholders' equity	<u>\$ 158,522</u>	<u>\$ 165,424</u>

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

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

	Three Months Ended March 31,	
	2016	2015
Revenue	\$ —	\$ 26
Operating expenses:		
Research and development	4,736	1,607
General and administrative	3,216	1,884
Total operating expenses	7,952	3,491
Loss from operations	(7,952)	(3,465)
Other income (expense):		
Interest income, net	104	—
Other income (expense), net	(4)	50
Total other income (expense), net	100	50
Loss before income tax provision	(7,852)	(3,415)
Income tax benefit (provision)	5	(50)
Net loss	\$ (7,847)	\$ (3,465)
Net loss per share attributable to ordinary shareholders—basic and diluted	\$ (0.36)	\$ (0.42)
Weighted-average ordinary shares used in computing net loss per share attributable to ordinary shareholders—basic and diluted	21,551,423	8,273,805

**WAVE LIFE SCIENCES LTD.**  
**UNAUDITED CONDENSED CONSOLIDATED STATEMENTS OF COMPREHENSIVE LOSS**

*(In thousands)*

	Three Months Ended March 31,	
	2016	2015
Net loss	\$ (7,847)	\$ (3,465)
Other comprehensive income (loss):		
Foreign currency translation	11	(28)
Comprehensive loss	\$ (7,836)	\$ (3,493)

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