WAVE Life Sciences Reports Fourth Quarter and Full-Year 2016 Financial Results and Provides Business Update

March 16, 2017

On track to take first three lead programs into the clinic in 2017

Clinical trials expected to commence in Huntington's disease mid-2017, Duchenne Muscular Dystrophy second half of 2017

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Mar. 16, 2017-- WAVE Life Sciences Ltd. (NASDAQ: WVE), a genetic medicines company focused on developing targeted therapies for patients impacted by rare diseases, today reported financial results for the fourth quarter and full year ended December 31, 2016, and provided a business update.

"Last year, we strengthened our foundation for growth and operational excellence in WAVE's core platform and neurology franchise, adding the leadership of Mike Panzara, M.D., M.P.H., and advancing our first three programs through to clinical candidates," said Paul Bolno, M.D., MBA, President and Chief Executive Officer of WAVE Life Sciences. "In 2017, we are poised to enter the clinic with our first three neurology programs and nominate the next three candidates, while also beginning production of drug supply in our cGMP manufacturing facility. At the same time, we will continue to leverage our R&D platform through our hepatic collaboration with Pfizer, and deepen our knowledge and capabilities in novel modalities such as single-stranded RNAi."

Recent Highlights and Outlook

- **Advancing three lead neurological programs into the clinic in 2017.** WAVE's two lead programs in Huntington's disease (HD) and one lead program in Duchenne Muscular Dystrophy (DMD) are expected to advance into clinical trials in 2017. WAVE has made significant progress towards this goal with all three candidates demonstrating strong preclinical data throughout 2016.

  Huntington's disease (HD): WVE-120101, WVE-120102

  WVE-120101 and WVE-120102 each target a distinct patient population, which together account for more than two-thirds of the HD population. Each therapeutic candidate is designed to selectively silence mRNA transcript produced by the disease-causing mutant huntingtin (HTT) allele in order to reduce the mutant HTT protein while leaving the healthy HTT mRNA transcript relatively intact to produce normal protein. Preclinical studies have been completed and both WVE-120101 and WVE-120102 are expected to enter clinical trials in mid-2017. WAVE expects to initiate two simultaneous Phase 1b/2a multi-ascending dose trials to evaluate the safety, tolerability, pharmacokinetics and pharmacodynamic effects, and potentially generate preliminary evidence of therapeutic effect in patients with early manifest HD.

  Duchenne Muscular Dystrophy (DMD): WVE-210201

  WAVE has selected its lead exon-skipping candidate, WVE-210201, to target deletions of Exon 51. Preclinical Western blot studies of WVE-210201 demonstrated 52% dystrophin protein restoration as compared with normal skeletal muscle tissue lysates, versus approximately 1% when testing other exon-skipping compounds. WAVE has initiated cGMP manufacturing, and is working closely with the patient community in development of its clinical trial protocol. WVE-210201 is expected to enter the clinic in the second half of 2017. The company intends to include both ambulatory and non-ambulatory patients, as well as patients previously treated with other exon-skipping compounds after appropriate washout in the trial protocol. Additional exon-skipping programs in preclinical development continue to advance.

- **Investing in R&D platform capabilities.** WAVE continues to leverage its proprietary platform to design and develop single, well-characterized medicines that have optimized pharmacology, address underserved patient populations and expand treatment options. WAVE's rational drug design process that produces stereopure oligonucleotides enables reduced use of phosphorothioate (PS) modifications, alongside the naturally occurring phosphodiester (PO), while retaining other important aspects of pharmacology.

  WAVE continues to advance its single-stranded RNAi technology for applications in metabolic disease and expects to expand opportunities to engage RNAi silencing mechanisms in tissues outside the liver. Additional therapeutic areas under development include ophthalmology and dermatology.

  Following promising preclinical distribution data in WAVE's CNS programs, and as WAVE's portfolio of programs expands into additional neurological disorders, WAVE is exploring the ability of chemical modifications to direct distribution and characterize productive uptake of nucleic acid cargo to specific cell-types and regions within the CNS. WAVE intends to select an additional three program candidates in 2017 and is on track to deliver six development programs by the end of 2018.

- **Developments in strategic partnerships.** In May 2016, WAVE entered a collaboration with Pfizer, focused on the

advancement of genetically defined targets for the treatment of metabolic diseases, bringing together WAVE's proprietary drug development platform, across antisense and single-stranded RNAi modalities, along with GalNAc and Pfizer's hepatic targeting technology for enhanced delivery to the liver. The company expects to present initial data and learnings from this collaboration in 2017. WAVE continues to make progress under this collaboration with three programs advancing through lead-optimization, including ApoC-III. The remaining two Pfizer collaboration programs are expected to be nominated by November 2017.

- **Establishing manufacturing leadership in oligonucleotides.** To provide internal cGMP manufacturing capabilities and increase control and visibility of our drug product supply chain, WAVE signed a lease in 2016 for a manufacturing facility of approximately 90,000 square feet in Lexington, MA that the company plans to occupy in late 2Q 2017. The company expects our internal expertise in cGMP manufacturing will support growth and secure availability of investigational drug product for current and future development activities and, potentially, commercial-scale manufacturing.

Fourth Quarter and Full Year 2016 Financial Results and Financial Guidance

WAVE reported a net loss of $18.5 million in the fourth quarter of 2016 compared to $7.1 million in fourth quarter of 2015. The company reported a net loss of $55.4 million for 2016 as compared to $19.2 million for the prior year. The increase in net loss for the fourth quarter and full year was largely due to increased research and development efforts as well as investments in the company's infrastructure.

Research and development expenses were $14.0 million for the fourth quarter of 2016 as compared to $3.5 million for the same period in 2015. Research and development expenses for the full year were $40.8 million as compared to $9.1 million for the prior year. The increase in research and development expenses for the fourth quarter and full year was primarily driven by increases in spending on third-party-related costs used to advance WAVE's pre-clinical and drug-discovery activities, as well as increases in salary and related benefits costs as our employee headcount grows.

General and administrative expenses were $5.2 million for the fourth quarter of 2016 as compared to $3.7 million for the same period in the prior year. General and administrative expenses were $16.0 million for the full year as compared to $10.4 million for the prior year. The increase in general and administrative expenses in the fourth quarter and full year was primarily due to increases in salary and benefits costs as our employee headcount grows, as well as increases in other general and administrative expenses.

WAVE ended 2016 with $150.3 million in cash and cash equivalents compared to $161.2 million as of December 31, 2015. The decrease in cash and cash equivalents was primarily the result of our net loss which was partially offset by payments received from Pfizer related to the collaboration agreement and the share purchase agreement which were entered into in May 2016.

The company expects that its capital resources available as of December 31, 2016, along with anticipated milestone payments under the existing collaboration, will fund 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 rationally redesigned 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.

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: the anticipated commencement of our clinical trials; the design and anticipated goals 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 stereopure therapies compared to other therapies; our advancing of therapies across multiple modalities and the anticipated benefits of that strategy; the anticipated timing and benefits of our internal manufacturing facility that we are building; our future growth; and the potential of our stereopure approach and nucleic acid therapeutics generally. 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 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 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 CONDENSED CONSOLIDATED BALANCE SHEETS

(In thousands, except share amounts)

	December 31, 2016	December 31, 2015
Assets		
Current assets:		
Cash and cash equivalents	$ 150,293	$ 161,220

Prepaid expenses and other current assets	1,483	146
Deferred tax assets	214	18
Total current assets	151,990	161,384
Property and equipment, net	8,607	2,789
Deferred tax assets	560	192
Restricted cash	3,601	1,055
Other assets	53	4
Total assets	$ 164,811	$ 165,424
Liabilities, Series A preferred shares and shareholders' equity		
Current liabilities:		
Accounts payable	$ 4,943	$ 2,811
Accrued expenses and other current liabilities	4,445	945
Current portion of capital lease obligation	62	62
Current portion of deferred revenue	2,705	—
Total current liabilities	12,155	3,818
Long-term liabilities:		
Capital lease obligation, net of current portion	16	78
Deferred revenue, net of current portion	8,311	—
Other liabilities	1,592	163
Total long-term liabilities	9,919	241
Total liabilities	$ 22,074	$ 4,059
Series A preferred shares, no par value; 3,901,348 shares issued and outstanding	7,874	7,874
Shareholders' equity:		
Ordinary shares, no par value; 23,502,169 and 21,551,423 shares issued and outstanding at December 31, 2016 and 2015, respectively	215,602	185,344
Additional paid-in capital	10,029	3,182
Accumulated other comprehensive income (loss)	(291)	41
Accumulated deficit	(90,477)	(35,076)
Total shareholders' equity	134,863	153,491
Total liabilities, Series A preferred shares and shareholders' equity	$ 164,811	$ 165,424

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UNAUDITED CONDENSED CONSOLIDATED STATEMENTS OF OPERATIONS

(In thousands, except share and per share amounts)

	For the Year Ended December 31,		
	2016	**2015**	**2014**
Revenue	$ 1,485	$ 152	$ —
Operating expenses:			
Research and development	40,818	9,057	2,395
General and administrative	15,994	10,393	2,999
Total operating expenses	56,812	19,450	5,394
Loss from operations	(55,327)	(19,298)	(5,394)
Other income (expense), net:			
Dividend income	255	—	—
Interest income (expense), net	337	86	(12)
Other income (expense), net	(50)	56	261
Total other income (expense), net	542	142	249
Loss before income tax provision	(54,785)	(19,156)	(5,145)
Income tax provision	(616)	(44)	(84)
Net loss	$ (55,401)	$ (19,200)	$ (5,229)
Net loss per share attributable to ordinary shareholders—basic and diluted	$ (2.43)	$ (1.83)	$ (1.34)
Weighted-average ordinary shares used in computing net loss per share attributable to ordinary shareholders—basic and diluted	22,800,628	10,501,455	3,911,556

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