Wave Life Sciences Reports First Quarter 2019 Financial Results and Provides Business Update

May 10, 2019

CAMBRIDGE, Mass., May 10, 2019 (GLOBE NEWSWIRE) -- Wave Life Sciences Ltd. (Nasdaq: WVE), a clinical-stage genetic medicines company committed to delivering life-changing treatments for people battling devastating diseases, today announced financial results for the first quarter ended March 31, 2019 and provided a business update.

“Since the start of the year, we have made significant progress advancing suvodirsen, our lead clinical program for the treatment of Duchenne muscular dystrophy patients amenable to exon 51 skipping, and we look forward to providing dystrophin biopsy data in the second half of the year. In parallel, initial commercialization activities are underway to support the potential approval and launch of this investigational therapy, first in the United States and then globally,” said Paul Bolno, MD, MBA, President and Chief Executive Officer of Wave Life Sciences. "Suvodirsen is the first of multiple development programs we intend to advance for Duchenne, potentially enabling us to reach more patients living with DMD. Beyond our Duchenne programs, we are advancing our differentiated PRECISION-HD clinical program, an allele-selective approach to treating Huntington’s disease, and we look forward to reporting topline clinical results from these HD studies by year-end.”

Business Update
Wave is committed to building a fully integrated genetic medicines company led by its clinical- and preclinical-stage programs for the treatment of neuromuscular, central nervous system and ophthalmologic diseases.

Neuromuscular Diseases

Initial commercialization activities ongoing following completion of suvodirsen Phase 1 clinical trial; efficacy data from ongoing open-label extension study expected this year

- In April 2019, Wave announced the final results from its Phase 1 clinical trial of investigational suvodirsen (WVE-210201) in boys with Duchenne muscular dystrophy (DMD) who are amenable to exon 51 skipping. The results demonstrated a favorable safety and tolerability profile of suvodirsen for continued clinical development in the ongoing open-label extension (OLE) study and planned Phase 2/3 clinical trial.

- Suvodirsen is currently being studied in an OLE study, initiated in August 2018 with patients from the Phase 1 clinical trial. Wave expects to deliver an interim analysis of dystrophin expression from muscle biopsies in boys receiving suvodirsen in this study in the second half of 2019.

- The company expects to file for an accelerated approval of suvodirsen in the United States in the second half of 2020, pending positive clinical dystrophin expression data.

DYSTANCE 51, Wave’s Phase 2/3 clinical trial of suvodirsen in DMD, intended to support global regulatory filings

- In April 2019, Wave announced the design of DYSTANCE 51, the planned Phase 2/3 efficacy and safety clinical trial of suvodirsen. The trial is designed to enroll boys who are between 5 and 12 years of age (inclusive) with a genetically confirmed diagnosis of DMD amenable to exon 51 skipping therapy. The DYSTANCE 51 primary efficacy endpoints will measure change in dystrophin protein level and change in the North Star Ambulatory Assessment score. In addition, the trial will include multiple functional outcome measures as secondary efficacy endpoints.

- In January 2019, the company announced that DYSTANCE 51 was selected for the U.S. Food and Drug Administration (FDA) complex innovative trial designs pilot program. Through this program, Wave intends to reduce the number of patients required to deliver conclusive clinical efficacy results, thereby minimizing the number of patients required in the placebo treatment arm and potentially accelerating study completion. Through participation in the program, the company has met with FDA staff to discuss design elements of the trial.

- DYSTANCE 51 is expected to be initiated in July 2019 and the company intends to use the results of this trial to seek regulatory approvals globally.

Aiming to bring meaningful dystrophin protein restoration to more patients living with DMD

- Wave is leveraging learnings from its ongoing DMD development and discovery efforts to advance WVE-N531, its preclinical candidate to treat DMD in boys amenable to exon 53 skipping. WVE-N531 induced up to 71% dystrophin protein restoration in DMD *in vitro* 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 exploring exon targets beyond those targeted by suvodirsen and WVE-N531, including exons 44, 45, 52, 54 and 55.

Central Nervous System (CNS) Diseases

Advancing PRECISION-HD clinical program, the first allele-selective approach for Huntington's disease patients

- Wave's 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 (HD), is continuing to enroll patients globally. The company expects to report topline clinical data from the PRECISION-HD program by the end of the year. These results are expected to include a summary of clinical safety results, the degree of mutant huntingtin protein lowering in cerebrospinal fluid (CSF) and the ratio of total huntingtin versus mutant huntingtin protein in CSF to assess wild-type huntingtin protein.

- WVE-120101 and WVE-120102, which selectively target the mutant allele of the *huntingtin* (*HTT*) gene, have been shown to reduce levels of mutant *HTT* mRNA and protein, while leaving wild-type or healthy *HTT* mRNA and protein largely intact in *in vitro* studies with patient-derived cell-lines. The healthy transcript is required to produce healthy HTT protein which is critical for neuronal function. Multiple preclinical studies in the literature indicate that long-term suppression of healthy HTT protein may have detrimental consequences. Wave's allele-specific approach may also enable the company to address the pre-manifest, or asymptomatic, HD patient population in the future.

Leveraging PRISM to optimize C9ORF72 program and potential future CNS candidates

- Wave announced today that it has further optimized its *C9ORF72*-targeting program in amyotrophic lateral sclerosis (ALS) and frontotemporal dementia (FTD) and is advancing a new lead candidate (WVE-C092), which preferentially targets the transcript containing the GGGGCC (G4C2) expansion in the *C9ORF72* gene. The company leveraged advances in PRISM, its discovery and drug development platform, to design a candidate with an improved profile, including a substantial increase in potency in a preclinical study over its prior lead candidate. The observed potency and the expected durability of WVE-C092 may allow dosing frequency to be substantially optimized.

- Subject to the submission of clinical trial applications and approval to proceed, the company would expect to initiate clinical development of WVE-C092 in the second half of 2020.

- The company is utilizing the learnings from PRISM to design additional stereopure oligonucleotides with optimized profiles across other CNS diseases as part of its ongoing collaboration with Takeda.

Ophthalmologic Diseases

- Wave continues to advance stereopure oligonucleotides for the potential treatment of inherited retinal diseases. Preclinical data demonstrated 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 clinical candidates that could achieve a therapeutic effect with only two doses per year. The company expects to announce its first ophthalmology candidate in the second half of 2019.

First Quarter 2019 Financial Results and Financial Guidance

Wave reported a net loss of $44.2 million in the first quarter of 2019 as compared to $35.2 million in the same period in 2018. The increase in net loss in the first quarter of 2019 was largely driven by increased research and development efforts and continued organizational growth to support Wave's corporate goals.

Research and development expenses were $40.1 million in the first quarter of 2019 as compared to $29.2 million in the same period in 2018. The increase in research and development expenses in the first quarter was primarily due to increased external expenses related to our suvodirsen clinical activities as well as increased investments in PRISM and other research and development expenses.

General and administrative expenses were $10.9 million in the first quarter of 2019 as compared to $8.0 million in the same period in 2018. The increase in general and administrative expenses in the first quarter was mainly driven by increases in employee headcount to support Wave's corporate goals, as well as increases in other general operating expenses.

As of March 31, 2019, Wave had $287.6 million in cash and cash equivalents as compared to $174.8 million as of December 31, 2018. The increase in cash and cash equivalents was mainly due to the $161.8 million in net proceeds from the January 2019 follow-on offering, partially offset by Wave's year-to-date net loss of $44.2 million.

Wave expects that its existing cash and cash equivalents, together with expected and committed cash from existing collaborations, will enable Wave to fund its operating and capital expenditure requirements to the end of 2020.

About PRISM ™

PRISM is Wave Life Sciences' proprietary discovery and drug development platform that enables genetically defined diseases to be targeted with stereopure oligonucleotides across multiple therapeutic modalities. PRISM combines the company's unique ability to construct stereopure oligonucleotides with a deep understanding of how the interplay among oligonucleotide sequence, chemistry and backbone stereochemistry impacts key pharmacological properties. By exploring these interactions through iterative analysis of in vitro and in vivo outcomes and artificial intelligence-driven predictive modeling, the company continues to define design principles that are deployed across programs to rapidly develop and manufacture clinical candidates that meet pre-defined product profiles.

About Wave Life Sciences

Wave Life Sciences (NASDAQ: WVE) is a clinical-stage genetic medicines company committed to delivering life-changing treatments for people battling devastating diseases. Wave aspires to develop best-in-class medicines across multiple therapeutic modalities using PRISM, the company's proprietary discovery and drug development platform that enables the precise design, optimization and production of stereopure oligonucleotides. Driven by a resolute sense of urgency, the Wave team is targeting a broad range of genetically defined diseases so that patients and families may realize a brighter future. To find out more, please visit www.wavelifesciences.com and follow Wave on Twitter @WaveLifeSci.

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; our identification of future candidates and their therapeutic potential; the anticipated therapeutic benefits of our potential therapies compared to others; our ability to design compounds using multiple modalities and the anticipated benefits of that model; the anticipated benefits of our proprietary manufacturing processes and our internal manufacturing facility; our future growth and anticipated transition to a fully integrated commercial-stage company; the potential benefits of PRISM and our stereopure oligonucleotides compared with stereorandom oligonucleotides; the benefit of 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 effectiveness of PRISM; the continued development and acceptance of oligonucleotides as a class of medicines; 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 contract research organizations, contract manufacturing organizations, collaborators and partners; our ability to manufacture or contract with third parties 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)

	March 31, 2019	December 31, 2018
Assets		
Current assets:		
Cash and cash equivalents	$ 287,567	$ 174,819
Current portion of accounts receivable	10,000	10,000
Prepaid expenses and other current assets	17,464	17,454
Total current assets	315,031	202,273
Long-term assets:		
Accounts receivable, net of current portion	50,000	50,000
Property and equipment, net	39,929	39,931
Operating lease right-of-use assets	19,333	—
Restricted cash	3,631	3,625
Other assets	2,688	111
Total long-term assets	115,581	93,667
Total assets	$ 430,612	$ 295,940
Liabilities, Series A preferred shares and shareholders' equity		

Current liabilities:		
Accounts payable	$ 14,577	$ 13,089
Accrued expenses and other current liabilities	8,490	14,736
Current portion of deferred rent	—	115
Current portion of deferred revenue	105,891	100,945
Current portion of lease incentive obligation	—	1,156
Current portion of operating lease liability	2,919	—
Total current liabilities	131,877	130,041
Long-term liabilities:		
Deferred rent, net of current portion	—	5,132
Deferred revenue, net of current portion	60,184	68,156
Lease incentive obligation, net of current portion	—	9,247
Operating lease liability, net of current portion	31,782	—
Other liabilities	2,039	2,142
Total long-term liabilities	94,005	84,677
Total liabilities	$ 225,882	$ 214,718
Series A preferred shares, no par value; 3,901,348 shares issued and outstanding at March 31, 2019 and December 31, 2018	$ 7,874	$ 7,874
Shareholders' equity:		
Ordinary shares, no par value; 34,255,406 and 29,472,197 shares issued and outstanding at March 31, 2019 and December 31, 2018, respectively	$ 538,414	$ 375,148
Additional paid-in capital	42,113	37,768
Accumulated other comprehensive income	250	153
Accumulated deficit	(383,921)	(339,721)
Total shareholders' equity	$ 196,856	$ 73,348
Total liabilities, Series A preferred shares and shareholders' equity	$ 430,612	$ 295,940

WAVE LIFE SCIENCES LTD.
UNAUDITED CONSOLIDATED STATEMENTS OF OPERATIONS AND COMPREHENSIVE LOSS

(In thousands, except share and per share amounts)

	Three Months Ended March 31,	
	2019	**2018**
Revenue	$ 3,026	$ 1,422
Operating expenses:		
Research and development	40,113	29,196
General and administrative	10,901	8,001
Total operating expenses	51,014	37,197
Loss from operations	(47,988)	(35,775)
Other income, net:		
Dividend income	1,424	356
Interest income, net	11	7
Other income, net	2,353	343
Total other income, net	3,788	706
Loss before income taxes	(44,200)	(35,069)
Income tax provision	—	(172)
Net loss	$ (44,200)	$ (35,241)
Net loss per share attributable to ordinary shareholders—basic and diluted	$ (1.36)	$ (1.26)
Weighted-average ordinary shares used in computing net loss per share attributable to ordinary shareholders—basic and diluted	32,597,158	27,919,063

Other comprehensive income (loss):		
Net loss	$ (44,200)	$ (35,241)
Foreign currency translation	97	49
Comprehensive loss	$ (44,103)	$ (35,192)

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