



Wave Life Sciences Reports First Quarter 2020 Financial Results and Provides Business Update

May 11, 2020

Data from both PRECISION-HD clinical trials expected in 2H 2020

Advancing clinical neurology pipeline – SNP3 and C9orf72 programs on track to initiate clinical development in 2H 2020

First ADAR-mediated RNA-editing data in non-human primates demonstrates editing efficiencies of up to 50%

Wave to host investor conference call and webcast at 8:30 a.m. ET today

CAMBRIDGE, Mass., May 11, 2020 (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, 2020 and provided a business update.

"During the last few months, our team has done a tremendous job of navigating the realities of the global COVID-19 pandemic, while making substantial progress on our critical priorities, including delivering on a key 2020 milestone, our initial *in vivo* RNA-editing dataset. We are working tirelessly to keep our clinical trials ongoing, our preclinical programs moving towards clinical development, and our discovery work on track, all while supporting each other and our communities during this difficult time," said Paul Bolno, MD, MBA, President and Chief Executive Officer of Wave Life Sciences.

"Our PRECISION-HD clinical trials of WVE-120101 and WVE-120102, two investigational compounds designed to selectively target mutant HTT for the treatment of Huntington's disease, are ongoing. While the pandemic has impacted several global clinical trial sites, the commitment of our patients and investigators remains resolute, speaking to the high unmet need for disease modifying therapeutics for this devastating disease. In addition, we currently remain on track to initiate clinical development for two additional neurological programs in the second half of 2020. Lastly, today we are announcing new data from our ADAR-mediated RNA-editing program and I look forward to sharing further updates on this emerging platform capability later this year."

Recent business highlights

PRECISION-HD programs for Huntington's disease (HD): Wave is developing a unique portfolio of investigational stereopure oligonucleotides designed to selectively target the mutant allele of the huntingtin (mHTT) gene, while leaving the wild-type (wtHTT) relatively intact.

PRECISION-HD2:

- The PRECISION-HD2 Phase 1b/2a clinical trial, Wave's clinical trial investigating WVE-120102, a stereopure oligonucleotide designed to selectively target the mHTT mRNA transcript that contains the SNP rs362331 (SNP2) for HD, is ongoing.
- Data from the 32 milligram (mg) dose cohort of the PRECISION-HD2 trial are currently expected in the second half of 2020.
- An open-label extension (OLE) study open to patients outside of the U.S. who participated in the Phase 1b/2a PRECISION-HD2 trial is ongoing.

PRECISION-HD1:

- In March 2020, Wave initiated a 32 mg dose cohort in the ongoing PRECISION-HD1 Phase 1b/2a clinical trial of WVE-120101, a stereopure oligonucleotide designed to selectively target the mHTT mRNA transcript that contains the SNP rs362307 (SNP1) for HD.
- Wave currently expects to deliver topline clinical data from the PRECISION-HD1 trial, including the 32 mg dose cohort, in the second half of 2020.
- An open-label extension (OLE) study open to patients outside of the U.S. who participated in the Phase 1b/2a PRECISION-HD1 trial is ongoing.

PRECISION-HD trials:

- Wave continues to work closely with the PRECISION-HD clinical trial sites to monitor the impact of the evolving COVID-19 pandemic. If global restrictions continue or worsen, the ability to evaluate patients in both of the PRECISION-HD trials as planned may be impacted.
- Wave is assessing the potential for a next higher dose cohort to be added to both PRECISION-HD trials.

SNP3 program for HD: Wave is advancing a third HD program, which is designed to selectively target an undisclosed SNP on the mHTT mRNA transcript (SNP3), while leaving the wild-type (wtHTT) relatively intact.

- Wave expects to initiate clinical development of its SNP3 program in the second half of 2020.

C9orf72 program for ALS and FTD: Wave's C9orf72 program is designed to selectively target the transcripts containing the hexanucleotide repeat expansion (G4C2) in the C9orf72 gene.

- Wave is advancing its C9orf72 preclinical program to potentially treat amyotrophic lateral sclerosis (ALS) and frontotemporal dementia (FTD) and expects to initiate clinical development in the second half of 2020.

Central nervous system (CNS) programs in collaboration with Takeda: Wave is leveraging its learnings from PRISM™ to design additional stereopure oligonucleotides with optimized profiles for CNS indications, including Parkinson's, Alzheimer's and others, as part of its ongoing collaboration with Takeda.

- In the first quarter, Wave achieved target validation *in vivo* with a lead compound for a second program and expects to achieve target validation for a third program in 2020.

RNA editing: Wave is designing a novel RNA-editing platform capability using endogenous ADAR (adenosine deaminases acting on RNA) enzymes via free uptake (non-viral, non-LNP) of RNA-editing oligonucleotides, which has the potential to be a best-in-class RNA-editing modality.

- Today, Wave announced it has achieved successful RNA editing of ACTB (Beta-actin) mRNA in non-human primates (NHPs) via endogenous ADARs using stereopure GalNAc-conjugated oligonucleotides. In an ongoing proof-of-concept study, Wave oligonucleotides demonstrated up to 50% A to I (G) editing of ACTB mRNA in the liver of NHPs two-days post-last dose. To the company's knowledge, these are the first publicly available data that demonstrate successful RNA editing *in vivo* in NHPs.
- This RNA-editing platform is expected to be applicable for a wide range of disease targets. Wave has previously shown that its RNA-editing oligonucleotides achieved editing across multiple distinct transcripts in primary human hepatocytes and this *in vitro* data will also be presented at the upcoming American Society of Gene & Cell Therapy (ASGCT) Annual Meeting, being held virtually May 12 – May 15.
- Wave expects to share additional *in vivo* ADAR-mediated RNA-editing data and to announce its first RNA-editing program in 2020.

First Quarter 2020 Financial Results and Financial Guidance

Wave reported a net loss of \$47.5 million in the first quarter of 2020 as compared to \$44.2 million in the same period in 2019.

Research and development expenses were \$41.2 million in the first quarter of 2020 as compared to \$40.1 million in the same period in 2019. The increase in research and development expenses in the first quarter was primarily due to increased external expenses related to our clinical and preclinical activities, including our HD programs and C9orf72 program for ALS and FTD, and separation costs associated with the workforce reduction implemented in February 2020, partially offset by decreased external expenses related to our DMD programs due to our December 2019 decision to discontinue the suvodirsen program and to cease development of our other DMD programs.

General and administrative expenses were \$13.0 million in the first quarter of 2020 as compared to \$10.9 million in the same period in 2019. The increase in general and administrative expenses in the first quarter of 2020 was mainly driven by separation costs associated with the workforce reduction implemented in February 2020.

As of March 31, 2020, Wave had \$120.9 million in cash and cash equivalents as compared to \$147.2 million as of December 31, 2019. The decrease in cash and cash equivalents was mainly due to Wave's year-to-date net loss of \$47.5 million, partially offset by the receipt of \$20 million in research support funding from Takeda under our collaboration.

Wave expects that its existing cash and cash equivalents, together with expected and committed cash from its existing collaboration, will enable Wave to fund its operating and capital expenditure requirements into the third quarter of 2021.

Investor Conference Call and Webcast

Wave management will host an investor conference call today at 8:30 a.m. ET to discuss the company's first quarter 2020 operating results and provide a business update. The conference call may be accessed by dialing (866) 220-8068 (domestic) or +1 (470) 495-9153 (international) and entering conference ID 5669348. The live webcast may be accessed from the investor relations section of the Wave Life Sciences corporate website at www.ir.wavelifesciences.com. Following the webcast, a replay will be available on the website.

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; regulatory submissions; 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 capabilities; 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 maintain the company infrastructure and personnel needed to achieve our goals; the impact of the COVID-19 global pandemic on our business 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 interactions; 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 our 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 indications, 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.

UNAUDITED CONSOLIDATED BALANCE SHEETS

(In thousands, except share amounts)

	<u>March 31, 2020</u>	<u>December 31, 2019</u>
Assets		
Current assets:		
Cash and cash equivalents	\$ 120,949	\$ 147,161
Current portion of accounts receivable	—	20,000
Prepaid expenses	9,999	9,626
Other current assets	18,843	8,689
Total current assets	<u>149,791</u>	<u>185,476</u>
Long-term assets:		
Accounts receivable, net of current portion	30,000	30,000
Property and equipment, net	34,986	36,368
Operating lease right-of-use assets	17,659	18,101
Restricted cash	3,649	3,647
Other assets	2,487	10,658
Total long-term assets	<u>88,781</u>	<u>98,774</u>
Total assets	<u>\$ 238,572</u>	<u>\$ 284,250</u>
Liabilities, Series A preferred shares and shareholders' equity		
Current liabilities:		
Accounts payable	\$ 16,486	\$ 9,073
Accrued expenses and other current liabilities	10,994	16,185
Current portion of deferred revenue	88,044	89,652
Current portion of operating lease liability	3,357	3,243
Total current liabilities	<u>118,881</u>	<u>118,153</u>
Long-term liabilities:		
Deferred revenue, net of current portion	60,913	63,466
Operating lease liability, net of current portion	28,425	29,304
Other liabilities	1,621	1,721
Total long-term liabilities	<u>\$ 90,959</u>	<u>\$ 94,491</u>
Total liabilities	<u>\$ 209,840</u>	<u>\$ 212,644</u>
Series A preferred shares, no par value; 3,901,348 shares issued and outstanding at March 31, 2020 and December 31, 2019	<u>\$ 7,874</u>	<u>\$ 7,874</u>
Shareholders' equity:		
Ordinary shares, no par value; 34,601,582 and 34,340,690 shares issued and outstanding at March 31, 2020 and December 31, 2019, respectively	\$ 540,161	\$ 539,547
Additional paid-in capital	61,276	57,277
Accumulated other comprehensive income	273	267
Accumulated deficit	(580,852)	(533,359)
Total shareholders' equity	<u>\$ 20,858</u>	<u>\$ 63,732</u>
Total liabilities, Series A preferred shares and shareholders' equity	<u>\$ 238,572</u>	<u>\$ 284,250</u>

The accompanying notes are an integral part of the unaudited consolidated financial statements.

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

(In thousands, except share and per share amounts)

	<u>Three Months Ended March 31,</u>	
	<u>2020</u>	<u>2019</u>
Revenue	\$ 4,161	\$ 3,026
Operating expenses:		
Research and development	41,158	40,113
General and administrative	12,996	10,901
Total operating expenses	<u>54,154</u>	<u>51,014</u>

Loss from operations	(49,993)	(47,988)
Other income, net:		
Dividend income	385	1,424
Interest income, net	3	11
Other income (expense), net	2,112	2,353
Total other income, net	<u>2,500</u>	<u>3,788</u>
Loss before income taxes	(47,493)	(44,200)
Income tax provision	<u>—</u>	<u>—</u>
Net loss	<u>\$ (47,493)</u>	<u>\$ (44,200)</u>
Net loss per share attributable to ordinary shareholders—basic and diluted	<u>\$ (1.38)</u>	<u>\$ (1.36)</u>
Weighted-average ordinary shares used in computing net loss per share attributable to ordinary shareholders—basic and diluted	<u>34,461,505</u>	<u>32,597,158</u>
Other comprehensive income (loss):		
Net loss	\$ (47,493)	\$ (44,200)
Foreign currency translation	6	97
Comprehensive loss	<u>\$ (47,487)</u>	<u>\$ (44,103)</u>

The accompanying notes are an integral part of the unaudited consolidated financial statements.

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