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

August 10, 2020

Data from 32 mg cohorts of both PRECISION-HD trials and PRECISION-HD OLE trials expected in 1Q 2021

C9orf72 and SNP3 clinical trial applications on track to be submitted in 4Q 2020

ADAR editing advancing with additional in vivo data expected at upcoming Analyst & Investor Research Webcast

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

CAMBRIDGE, Mass., Aug. 10, 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 second quarter ended June 30, 2020 and provided a business update.

"In the second quarter, despite persistent global challenges due to the COVID-19 pandemic, we continued to execute on our clinical and preclinical neurology programs. Our clinical, laboratory and manufacturing teams have proven to be adaptable and resilient as they work amidst new operating environments," said Paul Bolno, MD, MBA, President and Chief Executive Officer of Wave Life Sciences. "We are on track to submit clinical trial applications for our C9orf72 program for amyotrophic lateral sclerosis and frontotemporal dementia and our SNP3 program for HD in the fourth quarter of this year, both of which are designed with a novel chemistry application from our PRISM platform. During our upcoming Analyst & Investor Research Webcast on August 25, we plan to share exciting updates on our platform, including advances in oligonucleotide chemistry and our promising ADAR editing modality, and new preclinical data for our C9orf72 program."

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) protein relatively intact.

PRECISION-HD trials:

- The PRECISION-HD1 and PRECISION-HD2 Phase 1b/2a clinical trials evaluating investigational WVE-120101 and WVE-120102, stereopure oligonucleotides designed to selectively target the mHTT mRNA transcript that contains SNP rs362307 (SNP1) and rs362331 (SNP2), respectively, in patients with HD are ongoing.
- Wave expects to report data from the PRECISION-HD1 and PRECISION-HD2 trials, including the 32 mg dose cohorts for each trial, in the first quarter of 2021.
- Open-label extension (OLE) clinical trials for patients outside of the U.S. who participated in the Phase 1b/2a PRECISION-HD trials are ongoing, and data is expected to be reported in the first quarter of 2021.
- Wave continues to work closely with the PRECISION-HD clinical trial sites, which continue to face restrictions due to COVID-19.
- Wave is assessing the potential for a higher dose cohort to be added to both PRECISION-HD trials.

Publications:

- In May 2020, Wave's prospective observational study of the frequency of SNP1 and SNP2 in patients with HD was published in *Neurology Genetics*. As described in the manuscript titled "Genotyping single nucleotide polymorphisms for allele-selective therapy in Huntington's disease," the study confirms the feasibility of rapidly and prospectively identifying SNP1 and / or SNP2 in association with the mHTT allele in patients with HD, to enable allele-selective, personalized treatment approaches in eligible patients.

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

- Wave expects to initiate clinical development with the submission of a clinical trial application (CTA) for its SNP3 program in the fourth quarter of 2020.

C9orf72 program for amyotrophic lateral sclerosis (ALS) and frontotemporal dementia (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 ALS and FTD and expects to initiate clinical development with the submission of a CTA in the fourth quarter of 2020.

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

- To date, Wave has achieved target validation *in vivo* with a lead compound for two programs and expects to achieve target validation for a third program in 2020.

ADAR editing: Wave is advancing a novel RNA editing platform capability using endogenous ADAR (adenosine deaminases acting on RNA) enzymes via free uptake (non-viral, no nanoparticles) of A-to-I base editing oligonucleotides, which has the potential to be a best-in-class RNA editing modality.

- In May 2020, Wave presented at the American Society of Gene & Cell Therapy (ASGCT) Annual Meeting. The poster presentation highlighted data that demonstrated Wave's RNA editing oligonucleotides achieved editing across multiple distinct transcripts in primary human

hepatocytes *in vitro,* which suggests Wave's platform is applicable to a wide range of disease targets.
- Wave continues to advance its ADAR editing technology *in vitro* across multiple cell types and *in vivo* in multiple tissues. In the second quarter of 2020, Wave achieved successful editing of ACTB (Beta-actin) mRNA in non-human primates (NHPs) via endogenous ADARs using stereopure GalNAc-conjugated oligonucleotides. Wave expects to share additional *in vivo* ADAR editing data at its upcoming Analyst and Investor Research Webcast on August 25, 2020 and at scientific meetings in the second half of 2020.
- Wave also expects to announce its first ADAR editing program in 2020.

Strengthening leadership team: In May 2020, Wave appointed Kenneth Rhodes, PhD, as Senior Vice President, Therapeutics Discovery. Dr. Rhodes is responsible for defining the strategy and guiding discovery research to design new therapeutic candidates and advance them to the clinic, with an initial focus on neurological diseases.

Analyst and Investor Research Webcast: Wave is scheduled to hold an Analyst and Investor Research Webcast to discuss its latest PRISM platform advancements and neurology-focused oligonucleotide pipeline on Tuesday, August 25th, from 10:00 a.m. – 11:30 a.m.

- The webcast event will feature presentations from several members of Wave's management team, including President and CEO Paul Bolno, MD, MBA, who will present an update on Wave's strategy to become a leading genetic medicines company focused on neurology. Chandra Vargeese, PhD, Chief Technology Officer, will present an update on Wave's PRISM platform, novel chemistry advancements, and new data on Wave's ADAR editing platform capability. Kenneth Rhodes, PhD, Senior Vice President, Therapeutics Discovery, will present on Wave's current neurology pipeline, including its C9orf72 program for ALS and FTD, and opportunities to apply PRISM to address additional neurological diseases.

Second Quarter 2020 Financial Results and Financial Guidance

Wave reported a net loss of $40.5 million in the second quarter of 2020 as compared to $41.9 million in the same period in 2019.

Research and development expenses were $31.5 million in the second quarter of 2020 as compared to $41.6 million in the same period in 2019. The decrease in research and development expenses in the second quarter was primarily due to decreased external expenses related to suvodirsen due to our December 2019 decision to discontinue the program, partially offset by increased external expenses related to our clinical and preclinical activities, including our HD and C9orf72 programs for ALS and FTD.

General and administrative expenses were $10.2 million in the second quarter of 2020 as compared to $11.6 million in the same period in 2019. The decrease in general and administrative expenses in the second quarter of 2020 was mainly driven by decreased headcount resulting from the workforce reduction implemented in February 2020.

As of June 30, 2020, Wave had $94.1 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 $88 million, partially offset by the receipt of $20 million in research support funding from Takeda under our collaboration and $12 million in net proceeds under our at-the-market equity program.

Wave expects that its existing cash and cash equivalents, together with expected and committed cash from its existing collaboration, will enable the company to fund its operating and capital expenditure requirements into the fourth 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 second 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 4791316. The live webcast may be accessed from the investor relations section of the Wave Life Sciences corporate website at 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^{TM}, 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 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; the anticipated duration of our cash runway; and our expectations regarding the impact of the COVID-19 pandemic on our business. 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 severity and duration of the COVID-19 pandemic and its potentially negative impact on the conduct of, and the timing of enrollment, completion and reporting with respect to, our clinical trials; any other impacts on our business as a result of or related to the COVID-19 pandemic; 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 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.

WAVE LIFE SCIENCES LTD.
UNAUDITED CONSOLIDATED BALANCE SHEETS

(In thousands, except share amounts)

	June 30, 2020	December 31, 2019
Assets		
Current assets:		
Cash and cash equivalents	$ 94,054	$ 147,161
Current portion of accounts receivable	30,000	20,000
Prepaid expenses	6,452	9,626
Other current assets	16,328	8,689
Total current assets	146,834	185,476
Long-term assets:		
Accounts receivable, net of current portion	—	30,000
Property and equipment, net	33,096	36,368
Operating lease right-of-use assets	17,201	18,101
Restricted cash	3,650	3,647
Other assets	3,170	10,658
Total long-term assets	57,117	98,774
Total assets	$ 203,951	$ 284,250
Liabilities, Series A preferred shares and shareholders' equity		
Current liabilities:		
Accounts payable	$ 13,910	$ 9,073
Accrued expenses and other current liabilities	8,220	16,185
Current portion of deferred revenue	84,849	89,652
Current portion of operating lease liability	3,473	3,243
Total current liabilities	110,452	118,153
Long-term liabilities:		
Deferred revenue, net of current portion	61,081	63,466
Operating lease liability, net of current portion	27,513	29,304
Other liabilities	1,520	1,721
Total long-term liabilities	$ 90,114	$ 94,491
Total liabilities	$ 200,566	$ 212,644
Series A preferred shares, no par value; 3,901,348 shares issued and outstanding at June 30, 2020 and December 31, 2019	$ 7,874	$ 7,874
Shareholders' equity:		
Ordinary shares, no par value; 35,732,154 and 34,340,690 shares issued and outstanding at June 30, 2020 and December 31, 2019, respectively	$ 551,543	$ 539,547
Additional paid-in capital	65,070	57,277
Accumulated other comprehensive income	278	267
Accumulated deficit	(621,380)	(533,359)
Total shareholders' equity	$ (4,489)	$ 63,732
Total liabilities, Series A preferred shares and shareholders' equity	$ 203,951	$ 284,250

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)

	Three Months Ended June 30,		Six Months Ended June 30,	
	2020	2019	2020	2019
Revenue	$ 3,027	$ 7,628	$ 7,188	$ 10,654
Operating expenses:				
Research and development	31,478	41,605	72,636	81,718
General and administrative	10,205	11,640	23,201	22,541
Total operating expenses	41,683	53,245	95,837	104,259
Loss from operations	(38,656)	(45,617)	(88,649)	(93,605)
Other income (expense), net:				
Dividend income	135	1,544	520	2,968
Interest income (expense), net	(2)	8	1	19
Other income (expense), net	(2,005)	2,123	107	4,476
Total other income (expense), net	(1,872)	3,675	628	7,463
Loss before income taxes	(40,528)	(41,942)	(88,021)	(86,142)
Income tax provision	—	—	—	—
Net loss	$ (40,528)	$ (41,942)	$ (88,021)	$ (86,142)
Net loss per share attributable to ordinary shareholders —basic and diluted	$ (1.15)	$ (1.22)	$ (2.53)	$ (2.58)
Weighted-average ordinary shares used in computing net loss per share attributable to ordinary shareholders —basic and diluted	35,212,291	34,260,298	34,836,898	33,433,322
Other comprehensive income (loss):				
Net loss	$ (40,528)	$ (41,942)	$ (88,021)	$ (86,142)
Foreign currency translation	5	30	11	127
Comprehensive loss	$ (40,523)	$ (41,912)	$ (88,010)	$ (86,015)

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

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