

**UNITED STATES
SECURITIES AND EXCHANGE COMMISSION**

Washington, D.C. 20549

FORM 10-Q

(Mark One)

QUARTERLY REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934

For the quarterly period ended September 30, 2020

TRANSITION REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934

For the transition period from _____ to _____

Commission File Number: 001-37627

WAVE LIFE SCIENCES LTD.

(Exact name of registrant as specified in its charter)

Singapore

(State or other jurisdiction of incorporation or organization)

7 Straits View #12-00, Marina One East Tower

Singapore

(Address of principal executive offices)

Not applicable

(I.R.S. Employer Identification No.)

018936

(Zip Code)

+65 6236 3388

(Registrant's telephone number, including area code)

Securities registered pursuant to Section 12(b) of the Act:

Title of each class	Trading symbol	Name of each exchange on which registered
\$0 Par Value Ordinary Shares	WVE	The Nasdaq Global Market

Indicate by check mark whether the registrant: (1) has filed all reports required to be filed by Section 13 or 15(d) of the Securities Exchange Act of 1934 during the preceding 12 months (or for such shorter period that the registrant was required to file such reports), and (2) has been subject to such filing requirements for the past 90 days. Yes No

Indicate by check mark whether the registrant has submitted electronically every Interactive Data File required to be submitted pursuant to Rule 405 of Regulation S-T (§232.405 of this chapter) during the preceding 12 months (or for such shorter period that the registrant was required to submit such files). Yes No

Indicate by check mark whether the registrant is a large accelerated filer, an accelerated filer, a non-accelerated filer, a smaller reporting company, or an emerging growth company. See the definitions of "large accelerated filer," "accelerated filer," "smaller reporting company," and "emerging growth company" in Rule 12b-2 of the Exchange Act.

Large accelerated filer	<input checked="" type="checkbox"/>	Accelerated filer	<input type="checkbox"/>
Non-accelerated filer	<input type="checkbox"/>	Smaller reporting company	<input type="checkbox"/>
		Emerging growth company	<input type="checkbox"/>

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 13(a) of the Exchange Act.

Indicate by check mark whether the registrant is a shell company (as defined in Rule 12b-2 of the Exchange Act). Yes No

The number of outstanding ordinary shares of the registrant as of November 1, 2020 was 48,777,824.

WAVE LIFE SCIENCES LTD.
QUARTERLY REPORT ON FORM 10-Q
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Special Note Regarding Forward-Looking Statements

This Quarterly Report on Form 10-Q contains forward-looking statements within the meaning of Section 27A of the Securities Act of 1933, as amended, and Section 21E of the Securities Exchange Act of 1934, as amended, that relate to future events or to our future operations or financial performance. Any forward-looking statement involves known and unknown risks, uncertainties and other factors that may cause our actual results, levels of activity, performance or achievements to differ materially from any future results, levels of activity, performance or achievements expressed or implied by such forward-looking statement. In some cases, forward-looking statements are identified by the words “anticipate,” “believe,” “continue,” “could,” “estimate,” “expect,” “future,” “goals,” “intend,” “likely,” “may,” “might,” “ongoing,” “objective,” “plan,” “potential,” “predict,” “project,” “seek,” “should,” “strategy,” “target,” “will” and “would” or the negative of these terms, or other comparable terminology intended to identify statements about the future, although not all forward-looking statements contain these identifying words. Forward-looking statements include statements, other than statements of historical fact, about, among other things: our ability to fund our future operations; our financial position, revenues, costs, expenses, uses of cash and capital requirements; our need for additional financing or the period for which our existing cash resources will be sufficient to meet our operating requirements; the success, progress, number, scope, cost, duration, timing or results of our research and development activities, preclinical studies and clinical trials, including the timing for initiation or completion of or availability of results from any preclinical studies and clinical trials or for submission, review or approval of any regulatory filing; the timing of, and our ability to, obtain and maintain regulatory approvals for any of our product candidates; the potential benefits that may be derived from any of our product candidates; our strategies, prospects, plans, goals, expectations, forecasts or objectives; the success of our collaborations with third parties; any payment that our collaboration partners may make to us; our ability to identify and develop new product candidates; our intellectual property position; our commercialization, marketing and manufacturing capabilities and strategy; our ability to develop sales and marketing capabilities; our estimates regarding future expenses and needs for additional financing; our ability to identify, recruit and retain key personnel; our financial performance; developments and projections relating to our competitors in the industry; our liquidity and working capital requirements; the expected impact of new accounting standards; and our expectations regarding the impact of COVID-19 on our research and development activities, preclinical studies and clinical trials, supply of drug product, and our workforce.

Although we believe that we have a reasonable basis for each forward-looking statement contained in this report, we caution you that these statements are based on our estimates or projections of the future that are subject to known and unknown risks and uncertainties and other important factors that may cause our actual results, level of activity, performance or achievements expressed or implied by any forward-looking statement to differ. These risks, uncertainties and other factors include, among other things, our critical accounting policies and: the ability of our preclinical studies to produce data sufficient to support the filing of global 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 and timing of our programs, which may not support further development of our product candidates; actions of regulatory agencies, which may affect the initiation, timing and progress of clinical trials; our effectiveness in managing current and 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 fund our operations and to raise additional capital as needed; competition from others developing therapies for similar uses; the severity and duration of the COVID-19 pandemic; the COVID-19 pandemic may negatively impact 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, as well as other risks and uncertainties under the caption “Risk Factors” contained in this Quarterly Report on Form 10-Q and in other filings we make with the Securities and Exchange Commission.

Each forward-looking statement contained in this report is based on a combination of facts and factors currently known by us and our expectations of the future, about which we cannot be certain. As a result of these factors, we cannot assure you that the forward-looking statements in this report will prove to be accurate. Furthermore, if our forward-looking statements prove to be inaccurate, the inaccuracy may be material. In light of the significant uncertainties in these forward-looking statements, these statements should not be regarded as representations or warranties by us or any other person that we will achieve our objectives and plans in any specified timeframe, or at all. We caution you not to place undue reliance on any forward-looking statement.

In addition, any forward-looking statement in this report represents our views only as of the date of this report and should not be relied upon as representing our views as of any subsequent date. We anticipate that subsequent events and developments may cause our views to change. Although we may elect to update these forward-looking statements publicly at some point in the future, we undertake no obligation to publicly update any forward-looking statements, whether as a result of new information, future events or otherwise, except as required by applicable law. Our forward-looking statements do not reflect the potential impact of any future acquisitions, mergers, dispositions, joint ventures or investments we may make.

The Wave Life Sciences Ltd. and Wave Life Sciences Pte. Ltd. names, the Wave Life Sciences mark, PRISM and the other registered and pending trademarks, trade names and service marks of Wave Life Sciences Ltd. appearing in this Form 10-Q are the property of Wave Life Sciences Ltd. This Form 10-Q also contains additional trade names, trademarks and service marks belonging to Wave Life Sciences Ltd. and to other companies. We do not intend our use or display of other parties' trademarks, trade names or service marks to imply, and such use or display should not be construed to imply, a relationship with, or endorsement or sponsorship of us by, these other parties. Solely for convenience, the trademarks and trade names in this Form 10-Q are referred to without the ® and ™ symbols, but such reference should not be construed as any indicator that their respective owners will not assert, to the fullest extent under applicable law, their rights thereto.

PART I - FINANCIAL INFORMATION

Item 1. Financial Statements

**WAVE LIFE SCIENCES LTD.
UNAUDITED CONSOLIDATED BALANCE SHEETS**

(In thousands, except share amounts)

	<u>September 30, 2020</u>	<u>December 31, 2019</u>
Assets		
Current assets:		
Cash and cash equivalents	\$ 216,363	\$ 147,161
Current portion of accounts receivable	30,000	20,000
Prepaid expenses	7,966	9,626
Other current assets	4,101	8,689
Total current assets	<u>258,430</u>	<u>185,476</u>
Long-term assets:		
Accounts receivable, net of current portion	—	30,000
Property and equipment, net	31,116	36,368
Operating lease right-of-use assets	16,725	18,101
Restricted cash	3,650	3,647
Other assets	140	10,658
Total long-term assets	<u>51,631</u>	<u>98,774</u>
Total assets	<u>\$ 310,061</u>	<u>\$ 284,250</u>
Liabilities, Series A preferred shares and shareholders' equity		
Current liabilities:		
Accounts payable	\$ 9,699	\$ 9,073
Accrued expenses and other current liabilities	10,182	16,185
Current portion of deferred revenue	86,192	89,652
Current portion of operating lease liability	3,591	3,243
Total current liabilities	<u>109,664</u>	<u>118,153</u>
Long-term liabilities:		
Deferred revenue, net of current portion	56,288	63,466
Operating lease liability, net of current portion	26,574	29,304
Other liabilities	1,420	1,721
Total long-term liabilities	<u>\$ 84,282</u>	<u>\$ 94,491</u>
Total liabilities	<u>\$ 193,946</u>	<u>\$ 212,644</u>
Series A preferred shares, no par value; 3,901,348 shares issued and outstanding at September 30, 2020 and December 31, 2019	<u>\$ 7,874</u>	<u>\$ 7,874</u>
Shareholders' equity:		
Ordinary shares, no par value; 48,769,049 and 34,340,690 shares issued and outstanding at September 30, 2020 and December 31, 2019, respectively	\$ 694,066	\$ 539,547
Additional paid-in capital	68,354	57,277
Accumulated other comprehensive income	301	267
Accumulated deficit	(654,480)	(533,359)
Total shareholders' equity	<u>\$ 108,241</u>	<u>\$ 63,732</u>
Total liabilities, Series A preferred shares and shareholders' equity	<u>\$ 310,061</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 September 30,</u>		<u>Nine Months Ended September 30,</u>	
	<u>2020</u>	<u>2019</u>	<u>2020</u>	<u>2019</u>
Revenue	\$ 3,450	\$ 2,929	\$ 10,638	\$ 13,583
Operating expenses:				
Research and development	28,275	44,585	100,911	126,303
General and administrative	9,590	12,523	32,791	35,064
Total operating expenses	<u>37,865</u>	<u>57,108</u>	<u>133,702</u>	<u>161,367</u>
Loss from operations	(34,415)	(54,179)	(123,064)	(147,784)
Other income (expense), net:				
Dividend income	40	1,208	560	4,176
Interest income (expense), net	(17)	6	(16)	25
Other income, net	1,292	2,239	1,399	6,715
Total other income, net	<u>1,315</u>	<u>3,453</u>	<u>1,943</u>	<u>10,916</u>
Loss before income taxes	(33,100)	(50,726)	(121,121)	(136,868)
Income tax provision	—	—	—	—
Net loss	<u>\$ (33,100)</u>	<u>\$ (50,726)</u>	<u>\$ (121,121)</u>	<u>\$ (136,868)</u>
Net loss per share attributable to ordinary shareholders—basic and diluted	<u>\$ (0.86)</u>	<u>\$ (1.48)</u>	<u>\$ (3.36)</u>	<u>\$ (4.06)</u>
Weighted-average ordinary shares used in computing net loss per share attributable to ordinary shareholders—basic and diluted	<u>38,364,224</u>	<u>34,281,203</u>	<u>36,021,256</u>	<u>33,719,055</u>
Other comprehensive income (loss):				
Net loss	\$ (33,100)	\$ (50,726)	\$ (121,121)	\$ (136,868)
Foreign currency translation	23	2	34	129
Comprehensive loss	<u>\$ (33,077)</u>	<u>\$ (50,724)</u>	<u>\$ (121,087)</u>	<u>\$ (136,739)</u>

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

WAVE LIFE SCIENCES LTD.

UNAUDITED CONSOLIDATED STATEMENTS OF SERIES A PREFERRED SHARES AND SHAREHOLDERS' EQUITY

(In thousands, except share amounts)

	Series A Preferred Shares		Ordinary Shares		Additional Paid-In-Capital	Accumulated Other Comprehensive Income	Accumulated Deficit	Total Shareholders' Equity
	Shares	Amount	Shares	Amount				
Balance at December 31, 2018	3,901,348	\$ 7,874	29,472,197	\$ 375,148	\$ 37,768	\$ 153	\$ (339,721)	\$ 73,348
Issuance of ordinary shares, net of offering costs	—	—	4,542,500	161,785	—	—	—	161,785
Share-based compensation	—	—	—	—	4,345	—	—	4,345
Vesting of RSUs	—	—	110,187	—	—	—	—	—
Option exercises	—	—	130,522	1,481	—	—	—	1,481
Other comprehensive income	—	—	—	—	—	97	—	97
Net loss	—	—	—	—	—	—	(44,200)	(44,200)
Balance at March 31, 2019	<u>3,901,348</u>	<u>\$ 7,874</u>	<u>34,255,406</u>	<u>\$ 538,414</u>	<u>\$ 42,113</u>	<u>\$ 250</u>	<u>\$ (383,921)</u>	<u>\$ 196,856</u>
Issuance of ordinary shares	—	—	—	7	—	—	—	7
Share-based compensation	—	—	—	—	5,157	—	—	5,157
Option exercises	—	—	10,854	116	—	—	—	116
Other comprehensive income	—	—	—	—	—	30	—	30
Net loss	—	—	—	—	—	—	(41,942)	(41,942)
Balance at June 30, 2019	<u>3,901,348</u>	<u>\$ 7,874</u>	<u>34,266,260</u>	<u>\$ 538,537</u>	<u>\$ 47,270</u>	<u>\$ 280</u>	<u>\$ (425,863)</u>	<u>\$ 160,224</u>
Share-based compensation	—	—	—	—	5,020	—	—	5,020
Option exercises	—	—	17,957	253	—	—	—	253
Other comprehensive income	—	—	—	—	—	2	—	2
Net loss	—	—	—	—	—	—	(50,726)	(50,726)
Balance at September 30, 2019	<u>3,901,348</u>	<u>\$ 7,874</u>	<u>34,284,217</u>	<u>\$ 538,790</u>	<u>\$ 52,290</u>	<u>\$ 282</u>	<u>\$ (476,589)</u>	<u>\$ 114,773</u>

WAVE LIFE SCIENCES LTD.

UNAUDITED CONSOLIDATED STATEMENTS OF SERIES A PREFERRED SHARES AND SHAREHOLDERS' EQUITY CONTINUED

(In thousands, except share amounts)

	Series A Preferred Shares		Ordinary Shares		Additional Paid-In-Capital	Accumulated Other Comprehensive Income	Accumulated Deficit	Total Shareholders' Equity
	Shares	Amount	Shares	Amount				
Balance at December 31, 2019	3,901,348	\$ 7,874	34,340,690	\$ 539,547	\$ 57,277	\$ 267	\$ (533,359)	\$ 63,732
Issuance of ordinary shares pursuant to the at-the-market equity program, net	—	—	59,690	604	—	—	—	604
Share-based compensation	—	—	—	—	3,999	—	—	3,999
Vesting of RSUs	—	—	198,202	—	—	—	—	—
Option exercises	—	—	3,000	10	—	—	—	10
Other comprehensive income	—	—	—	—	—	6	—	6
Net loss	—	—	—	—	—	—	(47,493)	(47,493)
Balance at March 31, 2020	<u>3,901,348</u>	<u>\$ 7,874</u>	<u>34,601,582</u>	<u>\$ 540,161</u>	<u>\$ 61,276</u>	<u>\$ 273</u>	<u>\$ (580,852)</u>	<u>\$ 20,858</u>
Issuance of ordinary shares pursuant to the at-the-market equity program, net	—	—	1,123,156	11,372	—	—	—	11,372
Share-based compensation	—	—	—	—	3,794	—	—	3,794
Vesting of RSUs	—	—	3,569	—	—	—	—	—
Option exercises	—	—	3,847	10	—	—	—	10
Other comprehensive income	—	—	—	—	—	5	—	5
Net loss	—	—	—	—	—	—	(40,528)	(40,528)
Balance at June 30, 2020	<u>3,901,348</u>	<u>\$ 7,874</u>	<u>35,732,154</u>	<u>\$ 551,543</u>	<u>\$ 65,070</u>	<u>\$ 278</u>	<u>\$ (621,380)</u>	<u>\$ (4,489)</u>
Issuance of ordinary shares, net of offering costs	—	—	8,333,334	93,744	—	—	—	93,744
Issuance of ordinary shares pursuant to the at-the-market equity program, net	—	—	4,400,176	47,906	—	—	—	47,906
Share-based compensation	—	—	—	—	3,284	—	—	3,284
Vesting of RSUs	—	—	4,513	—	—	—	—	—
Option exercises	—	—	273,633	702	—	—	—	702
Issuance of ordinary shares under the ESPP	—	—	25,239	171	—	—	—	171
Other comprehensive income	—	—	—	—	—	23	—	23
Net loss	—	—	—	—	—	—	(33,100)	(33,100)
Balance at September 30, 2020	<u>3,901,348</u>	<u>\$ 7,874</u>	<u>48,769,049</u>	<u>\$ 694,066</u>	<u>\$ 68,354</u>	<u>\$ 301</u>	<u>\$ (654,480)</u>	<u>\$ 108,241</u>

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

WAVE LIFE SCIENCES LTD.
UNAUDITED CONSOLIDATED STATEMENTS OF CASH FLOWS

(In thousands)

	Nine Months Ended September 30,	
	2020	2019
Cash flows from operating activities		
Net loss	\$ (121,121)	\$ (136,868)
Adjustments to reconcile net loss to net cash used in operating activities:		
Amortization of right-of-use assets	1,376	1,187
Depreciation of property and equipment	6,097	5,584
Share-based compensation expense	11,077	14,522
Changes in operating assets and liabilities:		
Accounts receivable	20,000	10,000
Prepaid expenses	1,660	4,147
Other assets	15,106	(16,038)
Accounts payable	562	7,471
Accrued expenses and other current liabilities	(6,003)	(998)
Deferred revenue	(10,638)	(13,583)
Operating lease liabilities	(2,382)	(2,066)
Other non-current liabilities	(301)	(349)
Net cash used in operating activities	(84,567)	(126,991)
Cash flows from investing activities		
Purchases of property and equipment	(781)	(2,572)
Net cash used in investing activities	(781)	(2,572)
Cash flows from financing activities		
Proceeds from issuance of ordinary shares, net of offering costs	93,744	161,792
Proceeds from issuance of ordinary shares pursuant to the at-the-market equity program, net	59,882	—
Proceeds from the exercise of share options	722	1,850
Proceeds from the employee share purchase program	171	—
Net cash provided by financing activities	154,519	163,642
Effect of foreign exchange rates on cash, cash equivalents and restricted cash	34	129
Net increase in cash, cash equivalents and restricted cash	69,205	34,208
Cash, cash equivalents and restricted cash, beginning of period	150,808	178,444
Cash, cash equivalents and restricted cash, end of period	<u>\$ 220,013</u>	<u>\$ 212,652</u>

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

1. THE COMPANY

Organization

Wave Life Sciences Ltd. (together with its subsidiaries, “Wave” or the “Company”) is a clinical-stage genetic medicines company committed to delivering life-changing treatments for people battling devastating diseases. PRISM, Wave’s proprietary discovery and drug development platform, enables Wave to target genetically defined diseases with stereopure oligonucleotides across multiple therapeutic modalities.

The Company was incorporated in Singapore on July 23, 2012 and has its principal U.S. office in Cambridge, Massachusetts. The Company was incorporated with the purpose of combining two commonly held companies, Wave Life Sciences USA, Inc. (“Wave USA”), a Delaware corporation (formerly Ontorii, Inc.), and Wave Life Sciences Japan, Inc. (“Wave Japan”), a company organized under the laws of Japan (formerly Chiralgen., Ltd.), which occurred on September 13, 2012. On May 31, 2016, Wave Life Sciences Ireland Limited (“Wave Ireland”) was formed as a wholly-owned subsidiary of Wave Life Sciences Ltd. On April 3, 2017, Wave Life Sciences UK Limited (“Wave UK”) was formed as a wholly-owned subsidiary of Wave Life Sciences Ltd.

The Company’s primary activities since inception have been developing PRISM to design, develop and commercialize oligonucleotide therapeutics, advancing the Company’s neurology business, building the Company’s research and development activities in ophthalmology and hepatic, advancing programs into the clinic, furthering clinical development of such clinical-stage programs, building the Company’s intellectual property, and assuring adequate capital to support these activities.

Liquidity

Since its inception, the Company has not generated any product revenue and has incurred recurring net losses. To date, the Company has primarily funded its operations through private placements of debt and equity securities, public offerings of its ordinary shares and collaborations with third parties. Until the Company can generate significant revenue from product sales, if ever, the Company expects to continue to finance operations through a combination of public or private equity or debt financings or other sources, which may include collaborations with third parties. Adequate additional financing may not be available to the Company on acceptable terms, or at all. The inability to raise capital as and when needed would have a negative impact on the Company’s financial condition and ability to pursue its business strategy.

As of September 30, 2020, the Company had cash and cash equivalents of \$216.4 million. The Company expects that its existing cash and cash equivalents, together with expected and committed cash from its existing collaboration, will be sufficient to fund its operations for at least the next twelve months. The Company has based this expectation on assumptions that may prove to be incorrect, and the Company may use its available capital resources sooner than it currently expects. If the Company’s anticipated operating results are not achieved in future periods, planned expenditures may need to be further reduced in order to extend the time period over which the then-available resources would be able to fund the Company’s operations. In addition, the Company may elect to raise additional funds before it needs them if the conditions for raising capital are favorable due to market conditions or strategic considerations, even if the Company expects it has sufficient funds for its current or future operating plans.

Risks and Uncertainties

The Company is subject to risks common to companies in the biotechnology industry including, but not limited to, new technological innovations, protection of proprietary technology, maintaining internal manufacturing capabilities, dependence on key personnel, compliance with government regulations and the need to obtain additional financing. The Company’s therapeutic programs will require significant additional research and development efforts, including extensive preclinical and clinical testing and regulatory approval, prior to commercialization of any product candidates. These efforts require significant amounts of additional capital, adequate personnel infrastructure and extensive compliance-reporting capabilities. There can be no assurance that the Company’s research and development efforts will be successful, that adequate protection for the Company’s intellectual property will be obtained, that any products developed will obtain necessary government regulatory approval or that any approved products will be commercially viable. Even if the Company’s product development efforts are successful, it is uncertain when, if ever, the Company will generate significant revenue from product sales. The Company operates in an environment of rapid change in technology and substantial competition from pharmaceutical and biotechnology companies.

COVID-19 Global Pandemic

In December 2019, a novel strain of coronavirus was first identified in Wuhan, Hubei Province, China. Since that time, multiple other countries throughout the world and their economies have been effectively shut down and significantly affected by the spread of the virus. To date, responsive measures such as social distancing, work-from-home policies, travel bans and quarantines have been implemented in many countries throughout the world.

The Company is closely monitoring developments related to COVID-19, which was declared a pandemic by the World Health Organization on March 11, 2020. In response to this global pandemic, the Company has concentrated its efforts on the health and safety of its employees and patients, while maintaining business continuity and honoring its commitment to deliver life-changing treatments for people battling devastating diseases.

The COVID-19 global pandemic is evolving rapidly and its impact on the Company is highly uncertain and subject to change. The Company does not yet know the full extent of potential delays or long-term impacts on its business, its clinical trials, healthcare systems or the global economy. These impacts are highly uncertain and cannot be predicted with confidence, such as the geographic spread or resurgence of the disease, the duration of the outbreak, travel restrictions and actions to contain the outbreak or treat its impact, such as social distancing and quarantines or lock-downs, business closures or business disruptions and the effectiveness of actions taken to contain and treat the disease. These effects may materially adversely affect the Company's business, financial condition, results of operations, and prospects.

Basis of Presentation

The Company has prepared the accompanying consolidated financial statements in conformity with generally accepted accounting principles in the United States ("U.S. GAAP") and in U.S. dollars.

2. SIGNIFICANT ACCOUNTING POLICIES

The significant accounting policies described in the Company's audited financial statements as of and for the year ended December 31, 2019, and the notes thereto, which are included in the Company's Annual Report on Form 10-K for the year ended December 31, 2019, filed with the Securities and Exchange Commission ("SEC") on March 2, 2020, as amended (the "2019 Annual Report on Form 10-K"), have had no material changes during the three and nine months ended September 30, 2020.

Unaudited Interim Financial Data

The accompanying interim consolidated balance sheet as of September 30, 2020, the related interim consolidated statements of operations and comprehensive loss for the three and nine months ended September 30, 2020 and 2019, the consolidated statements of Series A preferred shares and shareholders' equity for the three months ended March 31, June 30 and September 30, 2020 and 2019, the consolidated statements of cash flows for the nine months ended September 30, 2020 and 2019, and the related interim information contained within the notes to the consolidated financial statements have been prepared in accordance with the rules and regulations of the SEC for interim financial information. Accordingly, they do not include all of the information and the notes required by U.S. GAAP for complete financial statements. The financial data and other information disclosed in these notes related to the three and nine months ended September 30, 2020 and 2019 are unaudited. In the opinion of management, the unaudited interim consolidated financial statements reflect all adjustments, consisting of normal and recurring adjustments, necessary for the fair presentation of the Company's financial position and results of operations for the three and nine months ended September 30, 2020 and 2019. The results of operations for the interim periods are not necessarily indicative of the results to be expected for the year ending December 31, 2020 or any other interim period or future year or period.

Principles of Consolidation

The Company's consolidated financial statements include the accounts of the Company and its wholly-owned subsidiaries. All intercompany balances and transactions have been eliminated in consolidation.

Reclassifications

The Company has reclassified certain prior period financial statement amounts to conform to its current period presentation. These reclassifications have not changed the results of operations of prior periods.

Recently Issued Accounting Pronouncements

The recently issued accounting pronouncements described in the Company's audited financial statements as of and for the year ended December 31, 2019, and the notes thereto, which are included in the 2019 Annual Report on Form 10-K, have had no material changes during the nine months ended September 30, 2020.

3. SHAREHOLDERS' EQUITY

September 2020 Follow-On Underwritten Public Offering

On September 25, 2020, the Company closed a follow-on underwritten public offering of 8,333,334 ordinary shares for gross proceeds of \$100.0 million (the "September 2020 Offering"). The estimated net proceeds to the Company from the September 2020 Offering are expected to be approximately \$93.7 million, after deducting underwriting discounts and commissions and estimated offering expenses.

At-The-Market Equity Program

The Company entered into an open market sales agreement with Jefferies LLC in May 2019, as amended in March 2020, for its "at-the-market" equity program. The Company first sold shares under the at-the-market equity program in 2020. During the nine months ended September 30, 2020, the Company sold 5,583,022 ordinary shares under its at-the-market equity program for aggregate net proceeds of \$59.9 million, after deducting commissions and offering expenses.

4. SHARE-BASED COMPENSATION

The Wave Life Sciences Ltd. 2014 Equity Incentive Plan, as amended (the "2014 Plan"), authorizes the board of directors or a committee of the board of directors to, among other things, grant non-qualified share options, restricted awards, which includes restricted shares, time-based restricted share units ("RSUs") and performance-based restricted share units ("PSUs") to eligible employees and directors of the Company. Options generally vest over periods of one to four years, and any options that are forfeited or cancelled are available to be granted again. The contractual life of options is generally five or ten years from the grant date. RSUs generally vest over a period of one to four years. PSUs vest upon the achievement of certain milestones. Any RSUs or PSUs that are forfeited are available to be granted again.

During the nine months ended September 30, 2020, the Company granted 959,685 options, including 50,000 options granted to an employee outside the 2014 Plan as an inducement material to entering into employment with the Company, in accordance with Nasdaq Listing Rule 5635(c)(4), to employees and non-employee directors and 38,800 RSUs to employees.

As of September 30, 2020, 2,215,440 ordinary shares remained available for future grant under the 2014 Plan.

Employee Share Purchase Plan

The Wave Life Sciences Ltd. Employee Share Purchase Plan ("ESPP") allows all full-time and certain part-time employees to purchase the Company's ordinary shares at a discount to fair market value. Eligible employees may enroll in a six-month offering period beginning every January 15th and July 15th. Shares are purchased at a price equal to 85% of the lower of the fair market value of the Company's ordinary shares on the first business day or the last business day of an offering period. Eligible employees who elected to participate in the ESPP were able to participate in the ESPP for the first time beginning on January 15, 2020. During the nine months ended September 30, 2020, 25,239 ordinary shares were issued under the ESPP. As of September 30, 2020, there were 974,761 ordinary shares available for issuance under the ESPP.

5. COLLABORATION AGREEMENTS

Pfizer Collaboration and Equity Agreements

In May 2016, the Company entered into a Research, License and Option Agreement (as amended in November 2017, the “Pfizer Collaboration Agreement”) with Pfizer Inc. (“Pfizer”). Pursuant to the terms of the Pfizer Collaboration Agreement, the Company and Pfizer agreed to collaborate on the discovery, development and commercialization of stereopure oligonucleotide therapeutics for up to five programs (the “Pfizer Programs”), each directed at a genetically-defined hepatic target selected by Pfizer (the “Pfizer Collaboration”). The Company received \$10.0 million as an upfront license fee under the Pfizer Collaboration Agreement. Subject to option exercises by Pfizer, the Company had the potential to earn research, development and commercial milestone payments, plus royalties, tiered up to low double-digits, on sales of any products that may result from the Pfizer Collaboration. None of the payments under the Pfizer Collaboration Agreement are refundable.

Simultaneously with the entry into the Pfizer Collaboration Agreement, the Company entered into a Share Purchase Agreement (the “Pfizer Equity Agreement,” and together with the Pfizer Collaboration Agreement, the “Pfizer Agreements”) with C.P. Pharmaceuticals International C.V., an affiliate of Pfizer (the “Pfizer Affiliate”). Pursuant to the terms of the Pfizer Equity Agreement, the Pfizer Affiliate purchased 1,875,000 of the Company’s ordinary shares (the “Shares”) at a purchase price of \$16.00 per share, for an aggregate purchase price of \$30.0 million. The Company did not incur any material costs in connection with the issuance of the Shares.

Under the Pfizer Collaboration Agreement, the parties agreed to collaborate during a four-year research term, which ended in May 2020. During the research term, the Company was responsible to use its commercially reasonable efforts to advance up to five programs through to the selection of clinical candidates. At that stage, Pfizer could elect to license any of these Pfizer Programs exclusively and obtain exclusive rights to undertake the clinical development of the resulting clinical candidates into products and the potential commercialization of any such products thereafter. In addition, the Company received a non-exclusive, royalty-bearing sublicensable license to use Pfizer’s hepatic targeting technology in any of the Company’s own hepatic programs that are outside the scope of the Pfizer Collaboration (the “Wave Programs”). If the Company used this technology on the Wave Programs, Pfizer would have been eligible to receive potential development and commercial milestone payments from the Company. Pfizer was also eligible to receive tiered royalties on sales of any products that include Pfizer’s hepatic targeting technology. The Company did not utilize Pfizer’s hepatic targeting technology in any of its own hepatic programs that are outside of the scope of the Pfizer Collaboration Agreement.

The stated term of the Pfizer Collaboration Agreement commenced on May 5, 2016 and terminated on the date of the last to expire payment obligation with respect to each Pfizer Program and, with respect to each Wave Program, expired on a program-by-program basis accordingly. Pfizer could terminate its rights related to a Pfizer Program under the Pfizer Collaboration Agreement at its own convenience upon 90 days’ notice to the Company. The Company could also terminate its rights related to a Wave Program at its own convenience upon 90 days’ notice to Pfizer. The Pfizer Collaboration Agreement could also be terminated by either party in the event of an uncured material breach of the Pfizer Collaboration Agreement by the other party.

Pfizer nominated two hepatic targets upon entry into the Pfizer Collaboration in May 2016. The Pfizer Collaboration Agreement provided Pfizer with options to nominate up to three additional programs by making nomination milestone payments. Pfizer nominated the third, fourth and fifth hepatic targets in August 2016, March 2018 and April 2018, respectively.

The Pfizer Collaboration was managed by a joint steering committee in which both parties were represented equally, which oversaw the scientific progression of each Pfizer Program up to the clinical candidate stage. During the four-year research term and for a period of two years thereafter, the Company agreed to work exclusively with Pfizer with respect to using any of the Company’s stereopure oligonucleotide technology that was specific for the applicable hepatic target which is the basis of any Pfizer Program. Within a specified period after receiving a data package for a candidate under each nominated program, Pfizer could exercise an option to obtain a license to develop, manufacture and commercialize the program candidate by paying an exercise price per program.

The Company assessed this arrangement in accordance with ASC 606 and concluded that the contract counterparty, Pfizer, was a customer. The Company identified the following promises under the arrangement: (1) the non-exclusive, royalty-free research and development license; (2) the research and development services for Programs 1 and 2; (3) the program nomination options for Programs 3, 4 and 5; (4) the research and development services associated with Programs 3, 4 and 5; (5) the options to obtain a license to develop, manufacture and commercialize Programs 1 and 2; and (6) the options to obtain a license to develop, manufacture and commercialize Programs 3, 4 and 5. The research and development services for each of Programs 1 and 2 were determined to not be distinct from the research and development license and should be combined into a single performance obligation for each program. The promises under the Pfizer Collaboration Agreement relate primarily to the research and development required by the Company for each of the programs nominated by Pfizer.

Additionally, the Company determined that the program nomination options for Programs 3, 4 and 5 were priced at a discount and, as such, provide material rights to Pfizer, representing three separate performance obligations. The research and development services associated with Programs 3, 4 and 5 and the options to obtain a license to develop, manufacture and commercialize Programs 3, 4 and 5 were subject to Pfizer's exercise of the program nomination options for such programs and therefore did not represent performance obligations at the outset of the arrangement. The options to obtain a license to develop, manufacture and commercialize Programs 1 and 2 did not represent material rights; as such, they were not representative of performance obligations at the outset of the arrangement. Based on these assessments, the Company identified five performance obligations in the Pfizer Collaboration Agreement: (1) research and development services and license for Program 1; (2) research and development services and license for Program 2; (3) material right provided for the option to nominate Program 3; (4) material right provided for the option to nominate Program 4; and (5) material right provided for the option to nominate Program 5.

At the outset of the arrangement, the transaction price included only the \$10.0 million up-front consideration received. The Company determined that the Pfizer Collaboration Agreement did not contain a significant financing component. The program nomination option exercise fees for research and development services associated with Programs 3, 4 and 5 that could be received are excluded from the transaction price until each customer option was exercised. The potential milestone payments were excluded from the transaction price, as all milestone amounts were fully constrained at the inception of the Pfizer Collaboration Agreement. The exercise fees for the options to obtain a license to develop, manufacture and commercialize Programs 3, 4 and 5 that could be received are excluded from the transaction price until each customer option was exercised. The Company reevaluated the transaction price at the end of each reporting period and as uncertain events were resolved or other changes in circumstances occurred, and, if necessary, adjusted its estimate of the transaction price.

During the year ended December 31, 2017, it became probable that a significant reversal of cumulative revenue would not occur for a developmental milestone under the Pfizer Collaboration Agreement. At such time, the associated consideration was added to the estimated transaction price and allocated to the existing performance obligations, and the Company recognized a cumulative catch-up to revenue for this developmental milestone, representing the amount that would have been recognized had the milestone payment been included in the transaction price from the outset of the arrangement. The remainder was recognized in the same manner as the remaining, unrecognized transaction price over the remaining period until each performance obligation was satisfied.

Revenue associated with the performance obligations relating to Programs 1 and 2 was recognized as revenue as the research and development services were provided using an input method, according to the full-time employee ("FTE") hours incurred on each program and the FTE hours expected to be incurred in the future to satisfy the performance obligation. The transfer of control occurred over time and, in management's judgment, this input method was the best measure of progress towards satisfying the performance obligation. The amount allocated to the three material rights was recognized as the underlying research and development services were provided commencing from the date that Pfizer exercised each respective option, or immediately as each option expired unexercised. The amounts received that had not yet been recognized as revenue were recorded in deferred revenue on the Company's consolidated balance sheet. As of September 30, 2020, there was no remaining deferred revenue related to the Pfizer Collaboration Agreement.

Pfizer nominated the third, fourth and fifth hepatic targets in August 2016, March 2018 and April 2018, respectively. Upon each exercise, the Company allocated the transaction price amount allocated to the material right at inception of the arrangement plus the program nomination option exercise fee paid by Pfizer at the time of exercising the option to a new performance obligation, which was recognized as revenue as the research and development services were provided using the same method as the performance obligations relating to Programs 1 and 2.

The research term for the Pfizer Collaboration Agreement ended in May 2020. Through September 30, 2020, the Company had recognized revenue of \$18.5 million as collaboration revenue in the Company's consolidated statements of operations and comprehensive loss under the Pfizer Collaboration Agreement. During the nine months ended September 30, 2020, the Company recognized revenue of \$1.5 million in the Company's consolidated statements of operations and comprehensive loss under the Pfizer Collaboration Agreement. During the three and nine months ended September 30, 2019, the Company recognized revenue of \$1.3 million and \$6.0 million, respectively, in the Company's consolidated statements of operations and comprehensive loss under the Pfizer Collaboration Agreement.

Takeda Collaboration and Equity Agreements

In February 2018, Wave USA and Wave UK entered into a global strategic collaboration (the "Takeda Collaboration") with Takeda Pharmaceutical Company Limited ("Takeda"), pursuant to which Wave USA, Wave UK and Takeda agreed to collaborate on the research, development and commercialization of oligonucleotide therapeutics for disorders of the Central Nervous System ("CNS"). The Takeda Collaboration provides Wave with at least \$230.0 million in committed cash and Takeda with the option to co-develop and co-commercialize Wave's CNS development programs in (1) Huntington's disease ("HD"); (2) amyotrophic lateral sclerosis ("ALS") and frontotemporal dementia ("FTD"); and (3) Wave's discovery-stage program targeting *ATXN3* for the treatment of spinocerebellar ataxia 3 ("SCA3") (collectively, "Category 1 Programs"). In addition, Takeda will have the right to exclusively license multiple preclinical programs for CNS disorders, including Alzheimer's disease and Parkinson's disease (collectively, "Category 2 Programs"). In April 2018, the Takeda Collaboration became effective and Takeda paid Wave \$110.0 million as an upfront payment. Takeda also agreed to fund Wave's research and preclinical activities in the amount of \$60.0 million during the four-year research term and to reimburse Wave for any collaboration-budgeted research and preclinical expenses incurred by Wave that exceed that amount.

Simultaneously with Wave USA and Wave UK's entry into the collaboration and license agreement with Takeda (the "Takeda Collaboration Agreement"), the Company entered into a share purchase agreement with Takeda (the "Takeda Equity Agreement," and together with the Takeda Collaboration Agreement, the "Takeda Agreements") pursuant to which it agreed to sell to Takeda 1,096,892 of its ordinary shares at a purchase price of \$54.70 per share. In April 2018, the Company closed the Takeda Equity Agreement and received aggregate cash proceeds of \$60.0 million. The Company did not incur any material costs in connection with the issuance of shares.

With respect to Category 1 Programs, Wave will be responsible for researching and developing products and companion diagnostics for Category 1 Programs through completion of the first proof of mechanism study for such products. Takeda will have an exclusive option for each target and all associated products and companion diagnostics for such target, which it may exercise at any time through completion of the proof of mechanism study. If Takeda exercises this option, Wave will receive an opt-in payment and will lead manufacturing and joint clinical co-development activities and Takeda will lead joint co-commercial activities in the United States and all commercial activities outside of the United States. Global costs and potential profits will be shared 50:50 and Wave will be eligible to receive development and commercial milestone payments. In addition to its 50% profit share, Wave is eligible to receive option exercise fees and development and commercial milestone payments for each of the Category 1 Programs.

With respect to Category 2 Programs, Wave has granted Takeda the right to exclusively license multiple preclinical programs during a four-year research term (subject to limited extension for programs that were initiated prior to the expiration of the research term, in accordance with the Takeda Collaboration Agreement) ("Category 2 Research Term"). During that term, the parties may collaborate on preclinical programs for up to six targets at any one time. Wave will be responsible for researching and preclinically developing products and companion diagnostics directed to the agreed upon targets through completion of Investigational New Drug application ("IND")-enabling studies in the first major market country. Thereafter, Takeda will have an exclusive worldwide license to develop and commercialize products and companion diagnostics directed to such targets, subject to Wave's retained rights to lead manufacturing activities for products directed to such targets. Takeda will fund Wave's research and preclinical activities in the amount of \$60.0 million during the research term and will reimburse Wave for any collaboration-budgeted research and preclinical expenses incurred by Wave that exceed that amount. Wave is also eligible to receive tiered high single-digit to mid-teen royalties on Takeda's global commercial sales of products from each Category 2 Program.

Under the Takeda Collaboration Agreement, each party grants to the other party specific intellectual property licenses to enable the other party to perform its obligations and exercise its rights under the Takeda Collaboration Agreement, including license grants to enable each party to conduct research, development and commercialization activities pursuant to the terms of the Takeda Collaboration Agreement.

The term of the Takeda Collaboration Agreement commenced on April 2, 2018 and, unless terminated earlier, will continue until the date on which: (i) with respect to each Category 1 Program target for which Takeda does not exercise its option, the expiration or termination of the development program with respect to such target; (ii) with respect to each Category 1 Program target for which Takeda exercises its option, the date on which neither party is researching, developing or manufacturing any products or companion diagnostics directed to such target; or (iii) with respect to each Category 2 Program target, the date on which royalties are no longer payable with respect to products directed to such target.

Takeda may terminate the Takeda Collaboration Agreement for convenience on 180 days' notice, in its entirety or on a target-by-target basis. Subject to certain exceptions, each party has the right to terminate the Takeda Collaboration Agreement on a target-by-target basis if the other party, or a third party related to such party, challenges the patentability, enforceability or validity of any patents within the licensed technology that cover any product or companion diagnostic that is subject to the Takeda Collaboration Agreement. In the event of any material breach of the Takeda Collaboration Agreement by a party, subject to cure rights, the other party may terminate the Takeda Collaboration Agreement in its entirety if the breach relates to all targets or on a target-by-target basis if the breach relates to a specific target. In the event that Takeda and its affiliates cease development, manufacturing and commercialization activities with respect to compounds or products subject to the Takeda Collaboration Agreement and directed to a particular target, Wave may terminate the Takeda Collaboration Agreement with respect to such target. Either party may terminate the Takeda Collaboration Agreement for the other party's insolvency. In certain termination circumstances, Wave would receive a license from Takeda to continue researching, developing and manufacturing certain products, and companion diagnostics.

The Takeda Collaboration is managed by a joint steering committee ("JSC") in which both parties are represented equally. The JSC is tasked with overseeing the scientific progression of each Category 1 Program and the Category 2 Programs.

The Company assessed this arrangement in accordance with ASC 606 and concluded that the contract counterparty, Takeda, is a customer for Category 1 Programs prior to Takeda exercising its option, and for Category 2 Programs during the Category 2 Research Term. The Company identified the following material promises under the arrangement: (1) the non-exclusive, royalty-free research and development license for each Category 1 Program; (2) the research and development services for each Category 1 Program through completion of the first proof of mechanism study; (3) the exclusive option to license, co-develop and co-commercialize each Category 1 Program; (4) the right to exclusively license the Category 2 Programs; and (5) the research and preclinical development services of the Category 2 Programs through completion of IND-enabling studies. The research and development services for each Category 1 Program were determined to not be distinct from the research and development license and should therefore be combined into a single performance obligation for each Category 1 Program. The research and preclinical development services for the Category 2 Programs were determined to not be distinct from the exclusive licenses for the Category 2 Programs and should therefore be combined into a single performance obligation.

Additionally, the Company determined that the exclusive option for each Category 1 Program was priced at a discount, and, as such, provide material rights to Takeda, representing three separate performance obligations. Based on these assessments, the Company identified seven performance obligations in the Takeda Collaboration Agreement: (1) research and development services through completion of the first proof of mechanism and non-exclusive research and development license for HD; (2) research and development services through completion of the first proof of mechanism and non-exclusive research and development license for ALS and FTD; (3) research and development services through completion of the first proof of mechanism and non-exclusive research and development license for SCA3; (4) the material right provided for the exclusive option to license, co-develop and co-commercialize HD; (5) the material right provided for the exclusive option to license, co-develop and co-commercialize ALS and FTD; (6) the material right provided for the exclusive option to license, co-develop and co-commercialize SCA3; and (7) the research and preclinical development services and right to exclusively license the Category 2 Programs.

At the outset of the arrangement, the transaction price included the \$110.0 million upfront consideration received and the \$60.0 million of committed research and preclinical funding for the Category 2 Programs. The Company determined that the Takeda Collaboration Agreement did not contain a significant financing component. The option exercise fees to license, co-develop and co-commercialize each Category 1 Program that may be received are excluded from the transaction price until each customer option is exercised. The potential milestone payments were excluded from the transaction price, as all milestone amounts were fully constrained at the inception of the Takeda Collaboration Agreement. The Company will reevaluate the transaction price at the end of each reporting period and as uncertain events are resolved or other changes in circumstances occur, if necessary, will adjust its estimate of the transaction price.

The Company allocated the transaction price to the performance obligations on a relative standalone selling price basis. For the performance obligations associated with the research and development services through completion of the first proof of mechanism and non-exclusive research and development license for HD; the research and development services through completion of the first proof of mechanism and non-exclusive research and development license for ALS and FTD; the research and development services through completion of the first proof of mechanism and non-exclusive research and development license for SCA3; and the research and preclinical development services and right to exclusively license the Category 2 Programs, the Company determined the standalone selling price using estimates of the costs to perform the research and development services, including expected internal and external costs for services and supplies, adjusted to reflect a profit margin. The total estimated cost of the research and development services reflected the nature of the services to be performed and the Company's best estimate of the length of time required to perform the services. For the performance obligations associated with the material right provided for the exclusive option to license, co-develop and co-commercialize HD; the material right provided for the exclusive option to license, co-develop and co-commercialize ALS and FTD; and the material right provided for the exclusive option to license, co-develop and co-commercialize SCA3, the Company estimated the standalone fair value of the option to license each Category 1 Program utilizing an adjusted market assessment approach, and determined that any standalone fair value in excess of the amounts to be paid by Takeda associated with each option represented a material right.

Revenue associated with the research and development services for each Category 1 Program performance obligation is being recognized as the research and development services are provided using an input method, according to the costs incurred on each Category 1 Program and the total costs expected to be incurred to satisfy each Category 1 Program performance obligation. Revenue associated with the research and preclinical development services for the Category 2 Programs performance obligation is being recognized as the research and preclinical development services are provided using an input method, according to the costs incurred on Category 2 Programs and the total costs expected to be incurred to satisfy the performance obligation. The transfer of control for these performance obligations occurs over time and, in management's judgment, this input method is the best measure of progress towards satisfying the performance obligations. The amount allocated to the material right for each Category 1 Program option will be recognized on the date that Takeda exercises each respective option, or immediately as each option expires unexercised. The amounts received that have not yet been recognized as revenue are recorded in deferred revenue on the Company's consolidated balance sheet.

Through September 30, 2020, the Company had recognized revenue of approximately \$27.5 million as collaboration revenue in the Company's consolidated statements of operations and comprehensive loss under the Takeda Collaboration Agreement. During the three and nine months ended September 30, 2020, the Company recognized revenue of \$3.4 million and \$9.1 million, respectively, in the Company's consolidated statements of operations and comprehensive loss under the Takeda Collaboration Agreement. During the three and nine months ended September 30, 2019, the Company recognized revenue of \$1.6 million and \$7.6 million, respectively, in the Company's consolidated statements of operations and comprehensive loss under the Takeda Collaboration Agreement. The aggregate amount of the transaction price allocated to the Company's unsatisfied and partially unsatisfied performance obligations and recorded in deferred revenue at September 30, 2020 is \$142.5 million, of which \$86.2 million is included in current liabilities. The Company expects to recognize revenue for the portion of the deferred revenue that relates to the research and development services for each Category 1 Program and the Category 2 Programs as costs are incurred, over the remaining research term. The Company expects to recognize revenue for the portion of the deferred revenue that relates to the material right for each Category 1 Program option upon Takeda's exercise of such option, or immediately as each option expires unexercised. The aggregate amount of the transaction price included in accounts receivable at September 30, 2020 is \$30.0 million, all of which is included in the current portion of accounts receivable.

6. NET LOSS PER ORDINARY SHARE

The Company applies the two-class method to calculate its basic and diluted net loss per share attributable to ordinary shareholders, as its Series A preferred shares are participating securities. The two-class method is an earnings allocation formula that treats a participating security as having rights to earnings that otherwise would have been available to ordinary shareholders.

Basic loss per share is computed by dividing net loss attributable to ordinary shareholders by the weighted-average number of ordinary shares.

The Company's potentially dilutive shares, which include outstanding share options to purchase ordinary shares, RSUs, PSUs and Series A preferred shares, are considered to be ordinary share equivalents and are only included in the calculation of diluted net loss per share when their effect is dilutive.

The following ordinary share equivalents, presented based on amounts outstanding at each period end, were excluded from the calculation of diluted net loss per share attributable to ordinary shareholders for the periods indicated because including them would have had an anti-dilutive effect:

	As of September 30,	
	2020	2019
Options to purchase ordinary shares	3,912,647	3,902,994
RSUs and PSUs	1,179,380	1,744,177
Series A preferred shares	3,901,348	3,901,348

Additionally, for the periods presented, the two-class method does not impact the net loss per ordinary share as the Company was in a net loss position for each of the periods presented and holders of Series A preferred shares do not participate in losses.

7. INCOME TAXES

During the three and nine months ended September 30, 2020 and 2019, the Company recorded no income tax provision.

The Company maintained a full valuation allowance for the three and nine months ended September 30, 2020 and 2019 in all jurisdictions due to uncertainty regarding future taxable income.

The Company's reserves related to taxes and its accounting for uncertain tax positions are based on a determination of whether and how much of a tax benefit taken by the Company in its tax filings or positions is more-likely-than-not to be realized following resolution of any potential contingencies present related to the tax benefit.

8. GEOGRAPHIC DATA

Substantially all of the Company's long-lived assets were located in the United States as of September 30, 2020 and December 31, 2019.

9. RELATED PARTIES

The Company had the following related party transaction for the periods presented in the accompanying consolidated financial statements:

- In 2012, the Company entered into a consulting agreement for scientific advisory services with Dr. Gregory L. Verdine, one of the Company's founders and a member of the Company's board of directors. The consulting agreement does not have a specific term and may be terminated by either party upon 14 days' prior written notice. Pursuant to the consulting agreement, the Company pays Dr. Verdine approximately \$13 thousand per month, plus reimbursement for certain expenses.

10. FEBRUARY 2020 COST REDUCTION PLAN

On February 6, 2020, the Company implemented a plan to reduce operating costs and better align its workforce with the needs of its business following the Company's December 16, 2019 announcement of its decision to discontinue the suvodirsen program for patients with Duchenne muscular dystrophy ("DMD"). Under this cost reduction plan, the Company reduced its workforce by approximately 22%. The Company incurred a one-time restructuring charge of approximately \$3.4 million, of which \$2.5 million was included in research and development expenses and \$0.9 million was included in general and administrative expenses in the Company's consolidated statements of operations and comprehensive loss, including employee severance, benefits and related termination costs, during the nine months ended September 30, 2020.

During the three and nine months ended September 30, 2020, the Company paid approximately \$0.2 million and \$3.1 million of these restructuring expenses, respectively. The remaining payments of \$0.3 million will be paid through March 31, 2021.

11. ACCRUED EXPENSES AND OTHER CURRENT LIABILITIES

Accrued expenses and other current liabilities consist of the following:

	<u>September 30, 2020</u>	<u>December 31, 2019</u>
	(in thousands)	
Accrued compensation	\$ 6,980	\$ 8,662
Accrued expenses related to CROs and CMOs	2,203	5,030
Accrued expenses and other current liabilities	999	2,493
Total accrued expenses and other current liabilities	<u>\$ 10,182</u>	<u>\$ 16,185</u>

Item 2. Management’s Discussion and Analysis of Financial Condition and Results of Operations

The following discussion and analysis of our financial condition and results of operations should be read in conjunction with our financial statements and related notes appearing elsewhere in this Quarterly Report on Form 10-Q and in our Annual Report on Form 10-K for the year ended December 31, 2019, filed with the Securities and Exchange Commission (“SEC”) on March 2, 2020, as amended (the “2019 Annual Report on Form 10-K”). Some of the information contained in this discussion and analysis or set forth elsewhere in this Quarterly Report on Form 10-Q, including information with respect to our plans and strategy for our business, includes forward-looking statements that involve risks and uncertainties. As a result of many factors, including those factors set forth in the “Risk Factors” section of this Quarterly Report on Form 10-Q, our actual results could differ materially from the results described in, or implied by, these forward-looking statements.

COVID-19 Business Update

We are closely monitoring developments related to COVID-19, which was declared a pandemic by the World Health Organization on March 11, 2020. In response to this global pandemic, we have concentrated our efforts on the health and safety of our employees and patients, while maintaining business continuity and honoring our commitment to deliver life-changing treatments for people battling devastating diseases.

We implemented business continuity plans in March 2020 designed to address the impact and potential impact of COVID-19 on our operations. Effective March 12, 2020, we implemented measures to mitigate the spread of COVID-19 and contribute to the ongoing public health effort to reduce the spread of the virus. We formed a COVID-19 Response Team to maintain business continuity while safeguarding employee and patient health. We also mandated a work-from-home policy for most employees and set up additional processes to work from home effectively, including measures to bolster our cybersecurity. Since early June 2020, we have been able to increase the number of employees working onsite, as well as expand the amount and type of manufacturing and lab-based activities being conducted onsite.

Our manufacturing operations and lab-based activities continue with social-distancing and updated protocols for accessing our facilities. As a biopharmaceutical research and development company, we are deemed to provide essential services under the “stay at home” advisory that was issued by the Governor of Massachusetts on March 23, 2020. While we continue to conduct R&D activities, including our ongoing clinical trials, the COVID-19 pandemic has impacted, and may continue to impact, certain of our early-stage discovery efforts and clinical trials. We are working with our clinical investigators, R&D vendors, and supply chain vendors to continually assess and take steps to mitigate the potential impact of COVID-19 on our manufacturing operations and R&D activities.

We will continue to closely monitor the COVID-19 situation as we evolve our business continuity plans. Given the global economic slowdown and the other risks and uncertainties associated with COVID-19, our business, financial condition, results of operations, and prospects could be materially adversely affected. For additional information, see “Item 1A. Risk Factors” of this Quarterly Report on Form 10-Q.

Overview

We are a clinical-stage genetic medicines company committed to delivering life-changing treatments for people battling devastating diseases. Using PRISM, our proprietary discovery and drug development platform that enables the precise design, optimization and production of novel stereopure oligonucleotides, we aspire to develop best in class medicines for genetically defined diseases with a high degree of unmet need.

Nucleic acid therapeutics, including oligonucleotides, are a growing and innovative class of drugs comprised of a sequence of nucleotides that are linked together by a backbone of chemical bonds. We are initially developing oligonucleotides that target the ribonucleic acid (“RNA”) to either reduce the expression of disease-promoting proteins or transform the production of dysfunctional mutant proteins into the production of functional proteins. RNA is a critical molecule that can adopt complex three-dimensional structures and affect various cellular functions. By intervening at the RNA level, we have the potential to address diseases that have historically been difficult to treat with small molecules or biologics. The mechanisms that we are currently using to target RNA with our oligonucleotides include RNase H-mediated RNA degradation, Ago2-mediated RNA interference (“RNAi”), exon-skipping, and ADAR (adenosine deaminases acting on RNA)-mediated RNA editing. Oligonucleotides have additional advantages as a therapeutic class including the ability to target multiple tissue types, often without the need for a delivery vehicle, and the ability to modulate the frequency of dosing to ensure broad distribution within tissues. Oligonucleotides also have well-established manufacturing processes and validated test methods based on decades of improvements.

The oligonucleotides we are developing with PRISM are stereopure. A stereopure oligonucleotide is comprised of molecules with atoms precisely arranged in three-dimensional orientations at each linkage. We believe that controlling the stereochemistry of each backbone position will optimize the pharmacological profile of our oligonucleotides by maximizing the potential therapeutic benefit while minimizing the potential for side effects and safety risks. The stereopure oligonucleotides we are developing differ from the mixture-based oligonucleotides currently on the market or in development by others. Our preclinical studies have demonstrated that our stereopure oligonucleotides may achieve superior pharmacological properties compared with mixture-based oligonucleotides. Through our work in developing stereopure oligonucleotides, we have created and continue to evolve PRISM, our proprietary discovery and drug development platform.

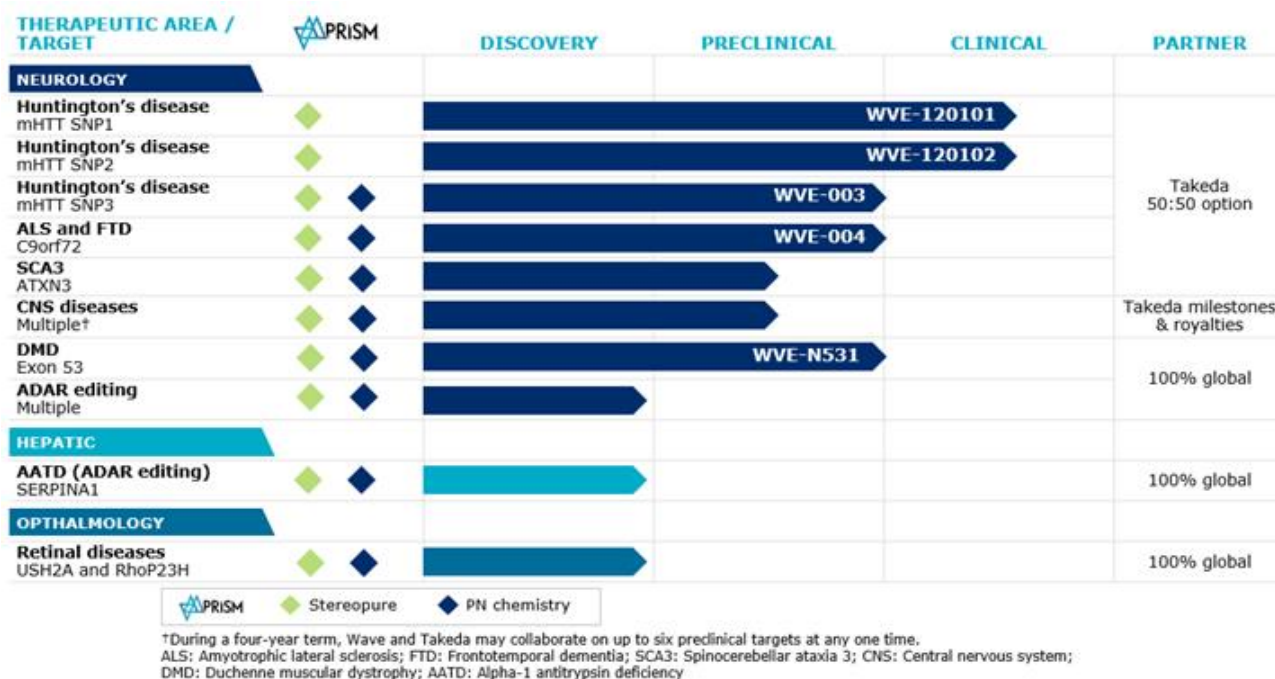
PRISM enables us to target genetically defined diseases with stereopure oligonucleotides across multiple therapeutic modalities. PRISM combines our unique ability to construct stereopure oligonucleotides with a deep understanding of how the interplay among oligonucleotide sequence, chemistry and backbone 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, we continue to define design principles that we deploy across programs to rapidly develop and manufacture clinical candidates that meet pre-defined product profiles.

During our Analyst and Investor Research Webcast on August 25, 2020, we shared the expansion of our repertoire of backbone modifications and their impact on oligonucleotide pharmacology with the introduction of Phosphoramidate diester (“PN”) backbone chemistry. PN chemistry is a backbone modification that involves replacing a non-bridging Oxygen atom with a Nitrogen-containing moiety. PN modifications are neutral, allowing them to break up the charge of the backbone while retaining specificity of complementary base pairings. Similar to the Phosphorothioate (“PS”) modification, PN modifications are also chiral. Data from our preclinical experiments demonstrate that the judicious use of PN backbone chemistry modifications in stereopure oligonucleotides have generally increased potency, exposure and durability across our silencing, splicing and editing modalities.

We have leveraged PN chemistry and our ability to develop stereopure oligonucleotides to advance our RNA-editing platform capability for the potential treatment of neurological and other diseases. Our novel approach uses endogenous ADAR (adenosine deaminases acting on RNA) enzymes via free uptake (non-viral, no nanoparticles) of A-to-I base editing oligonucleotides (“ADAR editing”). In a proof-of-concept study, we demonstrated successful RNA editing of ACTB (Beta-actin) mRNA in non-human primates (“NHPs”) using stereopure GalNAc-conjugated oligonucleotides. To our knowledge, these were the first publicly available data that demonstrated successful RNA editing *in vivo* in NHPs.

Our lead clinical development programs are focused in genetic diseases within neurology. Our most advanced stereopure therapeutic candidates in development, WVE-120101 and WVE-120102, are designed to selectively target mutant huntingtin (“mHTT”) and spare wild-type, or healthy, huntingtin (“wtHTT”) for the treatment of Huntington’s disease (“HD”). WVE-120101 and WVE-120102 are currently being studied in two Phase 1b/2a clinical trials, PRECISION-HD1 and PRECISION-HD2. Our next three neurology programs approaching clinical development, each of which incorporates PN backbone modifications, include our mHTT SNP3 program for the treatment of HD (WVE-003), our C9orf72 program for the treatment of amyotrophic lateral sclerosis (“ALS”) and frontotemporal dementia (“FTD”) (WVE-004), and our exon 53 program for Duchenne muscular dystrophy (“DMD”) (WVE-N531). We are also pursuing additional central nervous system (“CNS”) programs in collaboration with Takeda Pharmaceutical Company Limited (“Takeda”), including spinocerebellar ataxia 3 (“SCA3”). We are advancing discovery research in ADAR editing applications, including neurological, hepatic and lung diseases, and have identified alpha-1 antitrypsin deficiency (“AATD”) as our first ADAR editing program, which will target the SERPINA1 gene. In addition, outside of neurology, we are advancing discovery research in ophthalmologic disorders, specifically inherited retinal diseases. In further support of our pipeline, we continue to invest in PRISM to potentially develop the next generation of stereopure oligonucleotides. We have also established and continue to enhance

our internal current good manufacturing practices (“cGMP”) manufacturing capabilities to increase control and visibility of our drug substance supply chain.



Additional details regarding our programs are set forth below.

Neurology

Huntington's Disease ("HD"): HD is a rare hereditary neurodegenerative disease that results in early death and for which there is no cure. HD is caused by a mutation (i.e., an expanded CAG triplet repeat) in the HTT gene, which results in production of mutant HTT ("mHTT") protein. In HD patients, there is a progressive loss of neurons in the brain leading to cognitive, psychiatric and motor disabilities. HD patients still possess wild-type (healthy) HTT ("wtHTT") protein, which is important for neuronal function and there is increasing evidence that wtHTT may be neuroprotective in an adult brain. Additionally, a dominant gain of function in mHTT protein and a concurrent loss of function of wtHTT protein may be important components of the pathophysiology of HD. Accordingly, suppression of wtHTT may have detrimental long-term consequences. Absence of wtHTT protein has been shown to be embryonically lethal in mice. In October 2019, at our Analyst and Investor Research Day, key opinion leaders in HD research presented data suggesting that wtHTT is neuroprotective in an adult brain; transport of key neurotrophic factors such as brain-derived neurotrophic factor ("BDNF") are regulated by wtHTT levels; and HD may be caused by a dominant gain of function in mutant HTT and a loss of function of wtHTT protein. Further, the relative proportion of wtHTT to mHTT is critical based on evidence that suggests an increased amount of wtHTT relative to mHTT may result in slower disease progression (measured by age-at-onset). Also, HD patients that lack wtHTT all together have significantly more severe disease, as measured by disease progression after symptom onset.

Our HD Portfolio: In HD, we are currently advancing two clinical programs and one preclinical program. WVE-120101 and WVE-120102 are our clinical programs, where each is a distinct stereopure antisense oligonucleotide designed to selectively target a single nucleotide polymorphism ("SNP") associated with the disease-causing mutant huntingtin ("mHTT") mRNA transcript within the *HTT* gene: rs362307 ("mHTT SNP1") and rs362331 ("mHTT SNP2"), respectively. Our third program in HD, WVE-003, which we refer to as our "mHTT SNP3" program, is also a stereopure antisense oligonucleotide designed to selectively target an undisclosed SNP on the mHTT mRNA transcript, though it incorporates PN backbone modifications. We expect to initiate clinical development of WVE-003 with the submission of a CTA in the fourth quarter of 2020. Approximately 50% of the HD population carries SNP1 or SNP2 and, with overlap, up to 70% of the HD population carries either SNP1, SNP2 or both. Approximately 40% of the HD population carries SNP3 and, with overlap, up to 80% of the HD population carries at least one of SNP1, SNP2 and/or SNP3. Targeting mRNA transcript with these SNPs allows us to lower the mutant allele transcript, while leaving the healthy transcript relatively intact. The healthy transcript is required to produce healthy HTT protein which is important for neuronal function. We commonly refer to this method (or approach) as "allele selective targeting." SNPs are naturally occurring variations within a given genetic sequence and in certain instances can be used to distinguish between two related copies of a gene where only one is associated with the expression of a disease-causing protein. Our allele selective approach may also enable us to address the pre-manifest, or asymptomatic, HD patient

population in the future. We have shown that by targeting mHTT SNP1 and mHTT SNP2 in preclinical *in vitro* studies, the production of disease-causing proteins associated with HD can be selectively reduced. In addition, we have shown that by targeting mHTT SNP3 in preclinical *in vitro* studies, WVE-003 selectively reduces the expression of the mutant HTT.

SNP phasing technology: To verify that HD patients have at least one of the SNPs that we are targeting on the mutant allele, we investigated multiple technologies that could provide highly accurate results and rapid turnaround. We conducted a prospective observational study of the frequency of SNP1 and SNP2 in patients with HD, which confirmed the feasibility of rapidly and prospectively identifying SNP1 and / or SNP2 in association with the mHTT allele in patients with HD. This study was published in *Neurology Genetics* in May 2020 and the manuscript is titled, “Genotyping single nucleotide polymorphisms for allele-selective therapy in Huntington’s disease.” In addition, our SNP phasing methodology is summarized in *Molecular Therapy* in October 2020, with a manuscript titled, “Investigational Assay for Haplotype Phasing of the Huntingtin Gene.” In 2019, we entered into an agreement with Asuragen, Inc. (“Asuragen”), a molecular diagnostics company, for the development and potential commercialization of companion diagnostics for our investigational WVE-120101 and WVE-120102 allele-selective therapeutic programs in HD. We have since expanded our agreement with Asuragen to enable us to use their scalable SNP phasing technology in our upcoming clinical trial for WVE-003.

Phase 1b/2a Clinical Trials: PRECISION-HD is a global clinical program consisting of the PRECISION-HD1 and PRECISION-HD2 clinical trials. PRECISION-HD1 and PRECISION-HD2 are two parallel, multicenter, double-blind, randomized, placebo-controlled Phase 1b/2a clinical trials evaluating WVE-120101 and WVE-120102, respectively, administered intrathecally, consisting of single-ascending dose and multiple-ascending dose portions. The primary objective of these two trials is to assess the safety and tolerability of intrathecal doses of WVE-120101 and WVE-120102, respectively, in early manifest HD patients. Additional objectives include measurement of total HTT protein and mutant HTT protein, and exploratory pharmacokinetic, pharmacodynamic, clinical and MRI endpoints. Each trial is designed with five multi-dose cohorts (2, 4, 8, 16, and 32 mg), each with 12 patients that have Stage I or Stage II HD, ages 25-65, who have screened positively for the presence of SNP1 or SNP2. Outside of the United States, we are conducting both the single-ascending dose and multiple-ascending dose portions of the PRECISION-HD1 and PRECISION-HD2 trials. In the United States, we received approvals to proceed with the single-dose portions of both trials. However, the FDA indicated to us that we cannot progress to the multiple-ascending dose portions of these trials in the United States unless we conduct an additional preclinical study and present the resulting data to the FDA for its review. For the single-dose portion of the PRECISION-HD1 trial in the United States, escalation to our highest proposed doses is subject to the FDA’s review and approval of additional monitoring plans. WVE-120101 and WVE-120102 have been granted orphan drug designation for the treatment of HD by the FDA. In response to the global COVID-19 pandemic, we have taken, and will continue to take, actions to minimize disruptions to our PRECISION-HD clinical trials, including, among other actions, more frequent communications with our trial sites to monitor the impact of the evolving pandemic. As a result of the COVID-19 pandemic, our clinical trial sites have faced continued restrictions. If global restrictions continue or worsen, the ability to evaluate patients in both of the PRECISION-HD trials as planned may be further impacted.

PRECISION-HD2 trial: In December 2019, we announced initial clinical data from our ongoing PRECISION-HD2 trial. In an analysis comparing all patients treated with multiple intrathecal doses of WVE-120102 to placebo, a statistically significant reduction of 12.4% ($p < 0.05$) in mHTT protein was observed in cerebrospinal fluid (“CSF”). An analysis to assess a dose response across the initial four treatment groups (2, 4, 8, or 16 mg) suggested a statistically significant response in mHTT reduction at the highest doses tested ($p = 0.03$). WVE-120102 was generally safe and well tolerated across all cohorts. These topline data supported the addition of higher dose cohorts, and we initiated the 32 mg cohort in January 2020. We expect to deliver clinical data from the 32 mg cohort of the PRECISION-HD2 trial in the first quarter of 2021.

PRECISION-HD1 trial: We initiated the 32 mg cohort of the PRECISION-HD1 trial in March 2020. We expect to deliver clinical data from the five multi-dose cohorts (2, 4, 8, 16, 32 mg) of the PRECISION-HD1 trial in the first quarter of 2021.

Open-label Extensions of PRECISION-HD1 and PRECISION-HD2: In October 2019, we initiated an OLE of the PRECISION-HD2 trial outside of the United States for patients who participated in that trial. In February 2020, we initiated an OLE of the PRECISION-HD1 trial outside of the United States for patients who participated in that trial. We expect to report initial data from the PRECISION-HD OLE trials in the first quarter of 2021.

WVE-003 in HD: We expect to initiate clinical development of WVE-003, our mHTT SNP3 program, with the submission of a CTA in the fourth quarter of 2020.

WVE-004 in ALS and FTD: In amyotrophic lateral sclerosis (“ALS”) and frontotemporal dementia (“FTD”), we are advancing WVE-004, our C9orf72 program, which is designed to selectively target the transcripts containing the hexanucleotide repeat expansion (G4C2) in the C9orf72 gene. WVE-004, which incorporates PN backbone modifications, is designed to minimize the impact on normal C9orf72 protein in patients, thereby reducing potential on-target risk. The G4C2 expansion in the C9orf72 gene is the most common cause of familial ALS and FTD and is a strong genetic risk factor for non-inherited (sporadic) forms of ALS and FTD. We expect to initiate clinical development of WVE-004 with the submission of a CTA in the fourth quarter of 2020.

SCA3: In spinocerebellar ataxia 3 (“SCA3”), we are continuing to advance our program targeting ATXN3. SCA3 is a rare, hereditary (autosomal dominant), progressive, neurodegenerative disorder that is caused by a CAG-repeat expansion in the ATXN3 gene.

Additional CNS Disorders: We are collaborating with Takeda to advance genetically defined targets for the treatment of other CNS disorders, including Alzheimer’s disease, Parkinson’s disease, and others. Under the terms of the agreement, we may collaborate with Takeda on up to six preclinical programs at any one time, during a four-year term. Takeda is entitled to exclusively license multiple preclinical programs from us during the term.

Duchenne muscular dystrophy (DMD): DMD is a genetic disorder caused by mutations in the DMD gene that result in dysfunctional dystrophin protein. DMD impacts approximately one in every 5,000 newborn boys each year, resulting in approximately 20,000 new cases worldwide annually. In DMD, we are advancing WVE-N531, which targets exon 53, a region within the precursor messenger RNA (“pre-mRNA”) that is transcribed from the dystrophin gene (also referred to as the “DMD” gene). WVE-N531 incorporates PN backbone modifications and our planned clinical trial will enable us to assess the impact of PN chemistry on tissue distribution and splicing in dystrophic muscle. We expect to submit a CTA for WVE-N531 in the first quarter of 2021.

Hepatic and Pulmonary

We are leveraging our ADAR editing platform capability to develop a potentially novel treatment for alpha-1 antitrypsin deficiency (“AATD”). AATD is a rare, inherited genetic disorder that is commonly caused by a G-to-A point mutation in the SERPINA1 gene, called the *SERPINA1* Z allele. This mutation leads to misfolding and aggregation of alpha-1 antitrypsin (“AAT”) protein in hepatocytes and a lack of functional AAT in the lungs. People with AATD typically exhibit progressive lung damage, liver damage or both, leading to frequent hospitalizations and potentially terminal lung disease and/or liver disease. While the few approved therapies for AATD modestly increase circulating levels of AAT in those with the lung pathology, there are no approved therapies to address the liver pathology. Approximately 250,000 people worldwide are homozygous for the Z allele, which is the most severe form of the disease. Wave’s novel RNA editing platform capability uses endogenous ADAR enzymes of A-to-I (G) base editing oligonucleotides, making this a potentially best-in-class modality for correcting the G-to-A disease-causing mutation in mRNA coded by the *SERPINA1* Z allele. By correcting the single RNA base mutation, ADAR editing may provide an ideal approach for increasing circulating levels of wild-type AAT protein and reducing aggregation in the liver, thus simultaneously addressing both the lung and liver manifestations of the disease.

Ophthalmology

We are designing and advancing stereopure oligonucleotides for the potential treatment of rare, inherited eye diseases. Our preclinical data demonstrate that a single intravitreal injection of stereopure oligonucleotide in the eye of non-human primates (“NHPs”) resulted in greater than 95% knockdown of a target RNA in the retina for at least four months. Based on these data, we are working to design candidates that could achieve a therapeutic effect with only two doses per year. We are focused on advancing two preclinical programs: Usher syndrome type 2A (“USH2A”) and retinitis pigmentosa due to a P23H mutation in the RHO gene (“RhoP23H”). In October 2019, we presented *in vitro* and *ex vivo* preclinical data on our USH2A program, which is designed to promote USH2A exon 13 skipping, and we presented *in vitro* data on our RhoP23H program, which is designed to selectively silence RhoP23H transcripts.

Financial Operations Overview

We have never been profitable, and since our inception, we have incurred significant operating losses. Our net loss was \$121.1 million and \$136.9 million in the nine months ended September 30, 2020 and 2019, respectively. As of September 30, 2020 and December 31, 2019, we had an accumulated deficit of \$654.5 million and \$533.4 million, respectively. We expect to incur significant expenses and operating losses for the foreseeable future.

Revenue

We have not generated any product revenue since our inception and do not expect to generate any revenue from the sale of products for the foreseeable future. Our revenue during the nine months ended September 30, 2020 and 2019 represents revenue earned under our two revenue-generating collaboration agreements: the Pfizer Collaboration Agreement (as defined in Note 5 in the notes to the unaudited consolidated financial statements appearing elsewhere in this Quarterly Report on Form 10-Q (“Note 5”)), which was entered into in May 2016 and ended in May 2020, and the Takeda Collaboration Agreement (as defined in Note 5), which became effective in April 2018.

Operating Expenses

Our operating expenses since inception have consisted primarily of research and development costs and general and administrative costs.

Research and Development Expenses

Research and development expenses consist primarily of costs incurred for our research activities, including our discovery efforts, and the development of our product candidates, which include:

- compensation-related expenses, including employee salaries, bonuses, share-based compensation expense and other related benefits expenses for personnel in our research and development organization;
- expenses incurred under agreements with third parties, including contract research organizations (“CROs”) that conduct research, preclinical and clinical activities on our behalf, as well as contract manufacturing organizations (“CMOs”) that manufacture drug product for use in our preclinical studies and clinical trials;
- expenses incurred related to our internal manufacturing of drug substance for use in our preclinical studies and clinical trials;
- expenses related to compliance with regulatory requirements;
- expenses related to third-party consultants;
- research and development supplies and services expenses; and
- facility-related expenses, including rent, maintenance and other general operating expenses.

We recognize research and development costs as incurred. We recognize external development costs based on an evaluation of the progress to completion of specific tasks using information provided to us by our vendors. Payments for these activities are based on the terms of the individual agreements, which may differ from the pattern of costs incurred, and are reflected in our financial statements as prepaid or accrued expenses.

Our primary research and development focus since inception has been the development of our proprietary discovery and drug development platform, PRISM. We are using PRISM to design, develop and commercialize a broad pipeline of nucleic acid therapeutic candidates.

Our research and development expenses consist primarily of expenses related to our CROs, CMOs, consultants, other external vendors and fees paid to global regulatory agencies to conduct our clinical trials, in addition to compensation-related expenses, internal manufacturing expenses, facility-related expenses and other general operating expenses. These expenses are incurred in connection with research and development efforts and our preclinical studies and clinical trials. We track certain external expenses on a program-by-program basis. However, we do not allocate compensation-related expenses, internal manufacturing expenses, equipment repairs and maintenance expense, facility-related expenses or other operating expenses to specific programs. These expenses, which are not allocated on a program-by-program basis, are included in the “PRISM and other research and development expenses” category along with other external expenses related to our discovery and development programs, as well as platform development and identification of potential drug discovery candidates.

The table below summarizes our research and development expenses incurred for the three and nine months ended September 30, 2020 and 2019:

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2020	2019	2020	2019
	(in thousands)		(in thousands)	
DMD programs	\$ 464	\$ 10,848	\$ 6,531	\$ 35,856
HD programs	6,262	4,848	21,889	13,165
ALS and FTD programs	2,217	534	6,879	1,903
PRISM and other research and development expenses (1)	19,332	28,355	65,612	75,379
Total research and development expenses	\$ 28,275	\$ 44,585	\$ 100,911	\$ 126,303

- (1) Includes discovery and development programs, identification of potential drug discovery candidates, compensation-related expenses, internal manufacturing expenses, equipment repairs and maintenance expense, facility-related expenses and other operating expenses, which are not allocated to specific programs.

Product candidates in later stages of clinical development generally have higher development costs than those in earlier stages of clinical development, primarily due to the increased size and duration of later-stage clinical trials. We expect to continue to incur significant research and development expenses in the foreseeable future as we continue to manage our existing clinical trials, initiate additional clinical trials for certain product candidates, pursue later stages of clinical development for certain product candidates, maintain our manufacturing capabilities and continue to discover and develop additional product candidates in multiple therapeutic areas.

General and Administrative Expenses

General and administrative expenses consist primarily of compensation-related expenses, including salaries, bonuses, share-based compensation and other related benefits costs for personnel in our executive, finance, corporate, legal and administrative functions, as well as compensation-related expenses for our board of directors. General and administrative expenses also include legal fees; expenses associated with being a public company; professional fees for accounting, auditing, tax and consulting services; insurance costs; travel expenses; other operating costs; and facility-related expenses.

Other Income (Expense), Net

Other income (expense), net consists primarily of other income, net related to refundable tax credits from tax authorities and dividend income earned on cash and cash equivalents balances. We recognize refundable tax credits when there is reasonable assurance that we will comply with the requirements of the refundable tax credit and that the refundable tax credit will be received.

Income Taxes

We are a Singapore multi-national company subject to taxation in the United States and various other jurisdictions.

Critical Accounting Policies and Significant Judgments and Estimates

Our consolidated financial statements are prepared in accordance with generally accepted accounting principles in the United States. The preparation of our financial statements and related disclosures requires us to make estimates and assumptions that affect the reported amount of assets, liabilities, revenue, costs and expenses and related disclosures.

Our significant accounting policies, judgments and estimates are described in Note 2 in the notes to the audited consolidated financial statements included in the 2019 Annual Report on Form 10-K, as well as in Note 2 in the notes to the unaudited consolidated financial statements included in this Quarterly Report on Form 10-Q. We believe that our revenue recognition policy, particularly (a) assessing the number of performance obligations; (b) determining the transaction price; (c) allocating the transaction price to the performance obligations in the contract; and (d) determining the pattern over which performance obligations are satisfied, including estimates to complete performance obligations, and the assumptions and estimates used in our analysis of contracts with CROs and CMOs to estimate the contract expense, involve a greater degree of judgment, and therefore we consider them to be our critical accounting policies. We evaluate our estimates and assumptions on an ongoing basis. Our actual results may differ from these estimates under different assumptions and conditions.

Results of Operations

Comparison of the three months ended September 30, 2020 and 2019

	Three Months Ended September 30,		Change
	2020	2019	
	(in thousands)		
Revenue	\$ 3,450	\$ 2,929	\$ 521
Operating expenses:			
Research and development	28,275	44,585	(16,310)
General and administrative	9,590	12,523	(2,933)
Total operating expenses	<u>37,865</u>	<u>57,108</u>	<u>(19,243)</u>
Loss from operations	(34,415)	(54,179)	19,764
Total other income, net	<u>1,315</u>	<u>3,453</u>	<u>(2,138)</u>
Loss before income taxes	(33,100)	(50,726)	17,626
Income tax provision	—	—	—
Net loss	<u>\$ (33,100)</u>	<u>\$ (50,726)</u>	<u>\$ 17,626</u>

Revenue

Revenue of approximately \$3.4 million was earned under the Takeda Collaboration Agreement for the three months ended September 30, 2020 and revenue of \$2.9 million was earned under the Pfizer Collaboration Agreement and the Takeda Collaboration Agreement for the three months ended September 30, 2019. The \$0.5 million increase in revenue is due to an increase in research and development services under the Takeda Collaboration Agreement.

Research and Development Expenses

	Three Months Ended September 30,		Change
	2020	2019	
	(in thousands)		
DMD programs	\$ 464	\$ 10,848	\$ (10,384)
HD programs	6,262	4,848	1,414
ALS and FTD programs	2,217	534	1,683
PRISM and other research and development expenses (1)	19,332	28,355	(9,023)
Total research and development expenses	<u>\$ 28,275</u>	<u>\$ 44,585</u>	<u>\$ (16,310)</u>

(1) Includes discovery and development programs, identification of potential drug discovery candidates, compensation-related expenses, internal manufacturing expenses, equipment repairs and maintenance expense, facility-related expenses and other operating expenses, which are not allocated to specific programs.

Research and development expenses were \$28.3 million for the three months ended September 30, 2020, compared to \$44.6 million for the three months ended September 30, 2019. The decrease of \$16.3 million was due to the following:

- a decrease of \$10.4 million in external expenses related to our DMD programs, including suvodirsen, due to our December 2019 decision to discontinue the suvodirsen program;
- an increase of \$1.4 million in external expenses related to our HD programs, including costs related to our Phase 1b/2a clinical trials, PRECISION-HD1 and PRECISION-HD2, and our mHTT SNP3 program;
- an increase of \$1.7 million in external expenses related to our ALS and FTD programs; and
- a decrease of \$9.0 million in internal and external research and development expenses that are not allocated on a program-by-program basis and are related to other discovery and development programs, including PRISM and the identification of potential drug discovery candidates, primarily due to a decrease in supplies and services and a decrease in employee headcount due to the February 2020 reduction in workforce.

General and Administrative Expenses

General and administrative expenses were \$9.6 million for the three months ended September 30, 2020, as compared to \$12.5 million for the three months ended September 30, 2019. The decrease of \$2.9 million was attributable to a \$1.6 million decrease in compensation-related expenses and a \$1.3 million decrease in supplies and services expenses, both of which were primarily due to the February 2020 cost reduction plan, which included a workforce reduction.

Other Income (Expense), Net

Other income (expense), net for the three months ended September 30, 2020 and 2019 was \$1.3 million and \$3.5 million, respectively. The decrease of approximately \$2.1 million was driven by a \$1.2 million decrease in dividend income earned on cash and cash equivalents and a \$0.9 million decrease in other income, net primarily related to the decrease in the estimated refundable tax credit due to a decrease in the year-over-year underlying research and development expenses for the three months ended September 30, 2020.

Income Tax Provision

During the three months ended September 30, 2020 and 2019, we recorded no income tax provision. We maintained a full valuation allowance for the three months ended September 30, 2020 and 2019 in all jurisdictions due to uncertainty regarding future taxable income.

Comparison of the nine months ended September 30, 2020 and 2019

	Nine Months Ended September 30,		Change
	2020	2019	
	(in thousands)		
Revenue	\$ 10,638	\$ 13,583	\$ (2,945)
Operating expenses:			
Research and development	100,911	126,303	(25,392)
General and administrative	32,791	35,064	(2,273)
Total operating expenses	133,702	161,367	(27,665)
Loss from operations	(123,064)	(147,784)	24,720
Total other income, net	1,943	10,916	(8,973)
Loss before income taxes	(121,121)	(136,868)	15,747
Income tax provision	—	—	—
Net loss	\$ (121,121)	\$ (136,868)	\$ 15,747

Revenue

Revenue of approximately \$10.6 million and \$13.6 million was earned under the Pfizer Collaboration Agreement and the Takeda Collaboration Agreement for the nine months ended September 30, 2020 and 2019, respectively. The \$2.9 million decrease in revenue is primarily due to a decrease in research and development services under the Pfizer Collaboration Agreement, as the research term ended in May 2020.

Research and Development Expenses

	Nine Months Ended September 30,		Change
	2020	2019	
	(in thousands)		
DMD programs	\$ 6,531	\$ 35,856	\$ (29,325)
HD programs	21,889	13,165	8,724
ALS and FTD programs	6,879	1,903	4,976
PRISM and other research and development expenses (1)	65,612	75,379	(9,767)
Total research and development expenses	\$ 100,911	\$ 126,303	\$ (25,392)

- (1) Includes discovery and development programs, identification of potential drug discovery candidates, compensation-related expenses, internal manufacturing expenses, equipment repairs and maintenance expense, facility-related expenses and other operating expenses, which are not allocated to specific programs.

Research and development expenses were \$100.9 million for the nine months ended September 30, 2020, compared to \$126.3 million for the nine months ended September 30, 2019. The decrease of approximately \$25.4 million was due to the following:

- a decrease of \$29.3 million in external expenses related to our DMD programs, including suvodirsén, due to our December 2019 decision to discontinue the suvodirsén program;
- an increase of \$8.7 million in external expenses related to our HD programs, including costs related to our Phase 1b/2a clinical trials, PRECISION-HD1 and PRECISION-HD2, and our mHTT SNP3 program;
- an increase of \$5.0 million in external expenses related to our ALS and FTD programs; and
- a decrease of \$9.8 million in internal and external research and development expenses that are not allocated on a program-by-program basis and are related to other discovery and development programs, including PRISM and the identification of potential drug discovery candidates, primarily due to decreases in supplies and services, which was primarily due to the February 2020 cost reduction plan.

General and Administrative Expenses

General and administrative expenses were \$32.8 million for the nine months ended September 30, 2020, as compared to \$35.1 million for the nine months ended September 30, 2019. The decrease of \$2.3 million was mainly driven by a decrease in compensation-related expenses due to the decrease in employee headcount as a result of the February 2020 reduction in workforce.

Other Income (Expense), Net

Other income (expense), net for the nine months ended September 30, 2020 and 2019 was \$1.9 million and \$10.9 million, respectively. The decrease of \$9.0 million was driven by a \$5.3 million decrease in other income, net primarily related to the decrease in the estimated refundable tax credit due to a decrease in the year-over-year underlying research and development expenses and a \$3.6 million decrease in dividend income for the nine months ended September 30, 2020.

Income Tax Provision

During the nine months ended September 30, 2020 and 2019, we recorded no income tax provision. We maintained a full valuation allowance for the nine months ended September 30, 2020 and 2019 in all jurisdictions due to uncertainty regarding future taxable income.

Liquidity and Capital Resources

Since our inception, we have not generated any product revenue and have incurred recurring net losses. To date, we have primarily funded our operations through private placements of debt and equity securities, public offerings of our ordinary shares and collaborations with third parties. Through September 30, 2020, we have received an aggregate of approximately \$808.6 million in net proceeds from these transactions. We received \$89.3 million in net proceeds from private placements of our debt and equity securities, \$100.4 million in net proceeds from our initial public offering, \$40.0 million under the Pfizer Agreements (as defined in Note 5), including \$10.0 million as an upfront payment under the Pfizer Collaboration Agreement and \$30.0 million in the form of an equity investment, \$93.5 million in net proceeds from our April 2017 follow-on underwritten public offering, \$170.0 million in upfront payments under the Takeda Agreements (as defined in Note 5), including \$110.0 million as an upfront payment under the Takeda Collaboration Agreement (as defined in Note 5) and \$60.0 million in the form of an equity investment, \$161.8 million in net proceeds from our January 2019 follow-on underwritten public offering, \$59.9 million in net proceeds from our at-the-market equity program, and \$93.7 million in estimated net proceeds from our September 2020 follow-on underwritten public offering.

As of September 30, 2020, we had cash and cash equivalents totaling \$216.4 million, an accumulated deficit of \$654.5 million and restricted cash of \$3.7 million for our leased premises in Cambridge, Massachusetts and Lexington, Massachusetts.

We expect that our existing cash and cash equivalents, together with expected and committed cash from our existing collaboration, will be sufficient to fund our operations for at least the next twelve months. We have based this expectation on assumptions that may prove to be incorrect, and we may use our available capital resources sooner than we currently expect. In addition, we may elect to raise additional funds before we need them if the conditions for raising capital are favorable due to market conditions or strategic considerations, even if we expect we have sufficient funds for our current or future operating plans.

Until we can generate significant revenue from product sales, if ever, we expect to continue to finance our operations through a combination of public or private equity or debt financings or other sources, which may include collaborations with third parties.

In May 2019, we filed a shelf registration statement on Form S-3ASR with the SEC pursuant to which we registered for sale an indeterminate amount of any combination of our ordinary shares, debt securities, warrants, rights and/or units from time to time and at prices and on terms that we may determine. Our shelf registration statement on Form S-3ASR also includes a prospectus covering up to an aggregate of \$250.0 million in ordinary shares that we may issue and sell from time to time, through Jefferies LLC acting as our sales agent, pursuant to the open market sales agreement that we entered into with Jefferies LLC in May 2019, as amended in March 2020, for our at-the-market equity program. Since we no longer qualified as a “well-known seasoned issuer” at the time of the filing of our 2019 Annual Report on Form 10-K, we amended the shelf registration statement to register for sale up to \$500.0 million of any combination of our ordinary shares, debt securities, warrants, rights and/or units from time to time and at prices and on terms that we may determine, including the up to \$250.0 million in ordinary shares that we may issue and sell from time to time pursuant to our at-the-market equity program. During the nine months ended September 30, 2020, we sold 5,583,022 ordinary shares under our at-the-market equity program for aggregate gross proceeds of \$60.9 million and net proceeds of \$59.9 million, after deducting commissions and offering expenses. In addition, on September 25, 2020, we closed a follow-on underwritten public offering of 8,333,334 ordinary shares for gross proceeds of \$100.0 million. Estimated net proceeds to the Company from the offering were approximately \$93.7 million, after deducting underwriting discounts and commissions and estimated offering expenses. As of November 8, 2020, we have approximately \$339.1 million in securities available for sale under the shelf registration statement, including \$189.1 million in ordinary shares available for sale under the at-the-market equity program

Adequate additional financing may not be available to us on acceptable terms, or at all. Raising additional capital in the current economic environment, particularly in light of the economic downturn and ongoing uncertainty related to the COVID-19 pandemic, may be challenging. Our inability to raise capital as and when needed would have a negative impact on our financial condition and our ability to pursue our business strategy. We will need to generate significant revenue to achieve profitability, and we may never do so.

Cash Flows

The following table summarizes our cash flow activity:

	Nine Months Ended September 30,	
	2020	2019
	(in thousands)	
Net cash used in operating activities	\$ (84,567)	\$ (126,991)
Net cash used in investing activities	(781)	(2,572)
Net cash provided by financing activities	154,519	163,642
Effect of foreign exchange rates on cash, cash equivalents and restricted cash	34	129
Net increase in cash, cash equivalents and restricted cash	<u>\$ 69,205</u>	<u>\$ 34,208</u>

Operating Activities

During the nine months ended September 30, 2020, operating activities used \$84.6 million of cash, primarily due to our net loss of \$121.1 million, offset by a \$20.0 million decrease in accounts receivable and a \$15.1 million decrease in other assets, primarily related to the receipt of the refundable tax credits for 2018 and 2019.

During the nine months ended September 30, 2019, operating activities used \$127.0 million of cash, primarily due to our net loss of \$136.9 million.

Investing Activities

During the nine months ended September 30, 2020, investing activities used \$0.8 million of cash, related to purchases of property and equipment.

During the nine months ended September 30, 2019, investing activities used \$2.6 million of cash, related to purchases of property and equipment.

Financing Activities

During the nine months ended September 30, 2020, net cash provided by financing activities was \$154.5 million, primarily due to the \$93.7 million in estimated net proceeds from our September 2020 follow-on underwritten public offering and \$59.9 million in net proceeds from sales of ordinary shares under our at-the-market equity program.

During the nine months ended September 30, 2019, net cash provided by financing activities was \$163.6 million, which was due to the \$161.8 million in net proceeds from our January 2019 follow-on underwritten public offering and approximately \$1.9 million in proceeds from the exercise of share options.

Funding Requirements

We expect to continue to incur significant expenses in connection with our ongoing research and development activities and our internal cGMP manufacturing activities. Furthermore, we anticipate that our expenses will continue to vary if and as we:

- continue to conduct our clinical trials evaluating our product candidates in patients;
- conduct research and preclinical development of discovery targets and advance additional programs into clinical development;
- file clinical trial applications with global regulatory agencies and conduct clinical trials for our programs;
- make strategic investments in continuing to innovate our research and development platform, PRISM, and in optimizing our manufacturing processes and formulations;
- maintain our manufacturing capabilities through our internal facility and our CMOs;
- maintain our intellectual property portfolio and consider the acquisition of complementary intellectual property;

- seek and obtain regulatory approvals for our product candidates; and
- respond to the impacts of the COVID-19 global pandemic on our business.

We may experience delays or encounter issues with any of the above, including but not limited to failed studies, complex results, safety issues or other regulatory challenges.

Because of the numerous risks and uncertainties associated with the development of drug candidates and because the extent to which we may enter into collaborations with third parties for development of product candidates is unknown, we are unable to estimate the amounts of future capital outlays and operating expenses associated with completing the research and development for our therapeutic programs. Our future capital requirements for our therapeutic programs will depend on many factors, including:

- the progress, results and costs of conducting research and continued preclinical and clinical development for our therapeutic programs and future potential pipeline candidates;
- the number and characteristics of product candidates and programs that we pursue;
- the cost of manufacturing our product candidates;
- whether and to what extent milestone events are achieved under our collaboration with Takeda;
- the costs, timing and outcome of regulatory review of our product candidates;
- our ability to obtain marketing approval for our product candidates;
- the impacts of the COVID-19 global pandemic on our business;
- the costs and timing of future commercialization activities, including manufacturing, marketing, sales and distribution, for any of our product candidates for which we receive marketing approval;
- market acceptance of our product candidates, to the extent any are approved for commercial sale, and the revenue, if any, received from commercial sales of our product candidates for which we receive marketing approval;
- the costs and timing of preparing, filing and prosecuting patent applications, maintaining and enforcing our intellectual property rights and defending any intellectual property-related claims;
- the effect of competing technological and market developments; and
- the extent to which we acquire or invest in businesses, products and technologies, including entering into licensing or collaboration arrangements for product candidates.

Identifying potential product candidates and conducting preclinical testing and clinical trials is a time-consuming, expensive and uncertain process that takes years to complete, and we may never generate the necessary data or results required to obtain marketing approval and achieve product sales. In addition, our product candidates, if approved, may not achieve commercial success. Our product revenue, if any, will be derived from sales of products that we do not expect to be commercially available for many years, if ever. Accordingly, we will need to obtain substantial additional funds to achieve our business objectives.

Adequate additional funds may not be available to us on acceptable terms when we need them, or at all. We do not currently have any committed external source of funds, except for committed funds and possible future payments from Takeda under the Takeda Collaboration Agreement. To the extent that we raise additional capital through the sale of equity or convertible debt securities, the ownership interest of our existing shareholders will be diluted, and the terms may include liquidation or other preferences that adversely affect the rights of our shareholders. Additional debt financing and preferred equity financing, if available, may involve agreements that include covenants limiting or restricting our ability to take specific actions, such as incurring additional debt, making capital expenditures or declaring dividends and may require the issuance of warrants, which could potentially dilute our shareholders' ownership interests.

If we raise additional funds through collaborations, strategic alliances or licensing arrangements with third parties, we may have to relinquish valuable rights to our technologies, future revenue streams, research programs or product candidates or grant licenses on terms that may not be favorable to us. If we are unable to raise additional funds through equity or debt financings when needed, we may be required to delay, limit, reduce or terminate our product development programs or any future commercialization efforts or grant rights to develop and market product candidates that we would otherwise prefer to develop and market ourselves.

Contractual Obligations and Commitments

There have been no material changes to our contractual obligations and commitments set forth under the heading "Management's Discussion and Analysis of Financial Condition and Results of Operations – Contractual Obligations and Commitments" in our 2019 Annual Report on Form 10-K.

Off-Balance Sheet Arrangements

We had no off-balance sheet arrangements (as that term is defined in Item 303(a)(4)(ii) of Regulation S-K) as of September 30, 2020 that have, or are reasonably likely to have, a current or future effect on our financial condition, changes in financial condition, revenues or expenses, results of operations, liquidity, capital expenditures or capital resources.

Recently Issued Accounting Pronouncements

The recently issued accounting pronouncements described in the Company's audited financial statements as of and for the year ended December 31, 2019, and the notes thereto, which are included in the 2019 Annual Report on Form 10-K, have had no material changes during the nine months ended September 30, 2020.

Item 3. Quantitative and Qualitative Disclosures About Market Risk

Market risk represents the risk of loss that may impact our financial position due to adverse changes in financial market prices and rates. Our market risk exposure is primarily the result of fluctuations in interest rates and foreign exchange rates, as well as, to a lesser extent, inflation and capital market risk.

Interest Rate Risk

We are exposed to interest rate risk in the ordinary course of our business. Our cash and cash equivalents are held in readily available checking and money market accounts.

Foreign Currency Exchange Rate Risk

Due to our operations outside of the United States, we are exposed to market risk related to changes in foreign currency exchange rates. Historically, we have not hedged our foreign currency exposure. For the three and nine months ended September 30, 2020 and 2019, changes in foreign currency exchange rates did not have a material impact on our business, financial condition, results of operations or cash flows.

Inflation Risk

We do not believe that inflation had a material effect on our business, financial condition, results of operations or cash flows for the three and nine months ended September 30, 2020 and 2019.

Capital Market Risk

We currently have no product revenues and depend on funds raised through other sources. One possible source of funding is through further equity offerings. Our ability to raise funds in this manner depends upon capital market forces affecting our share price, including impacts on the capital markets resulting from the COVID-19 pandemic.

Item 4. Controls and Procedures

Evaluation of Disclosure Controls and Procedures

Our management, with the participation of our principal executive officer and principal financial officer, evaluated the effectiveness of our disclosure controls and procedures as of September 30, 2020. The term "disclosure controls and procedures," as defined in Rules 13a-15(e) and 15d-15(e) under the Securities Exchange Act of 1934, as amended (the "Exchange Act"), means controls and other procedures of a company that are designed to ensure that information required to be disclosed by a company in the reports that it files or submits under the Exchange Act is recorded, processed, summarized and reported, within the time periods specified in the SEC's rules and forms. Disclosure controls and procedures include, without limitation, controls and procedures designed to ensure that information required to be disclosed by a company in the reports that it files or submits under the Exchange Act is accumulated and communicated to its management, including its principal executive and principal financial officers, as appropriate to allow timely decisions regarding required disclosure. Management recognizes that any controls and procedures, no matter how well designed and operated, can provide only reasonable assurance of achieving their objectives and management necessarily applies its judgment in evaluating the cost-benefit relationship of possible controls and procedures. Based on the evaluation of our disclosure controls and procedures as of September 30, 2020, our principal executive officer and principal financial officer concluded that, as of such date, our disclosure controls and procedures were effective at the reasonable assurance level.

Changes in Internal Control over Financial Reporting

There were no changes in our internal control over financial reporting identified in connection with the evaluation of such internal control required by Rule 13a-15(d) and 15d-15(d) of the Exchange Act that occurred during the three months ended September 30, 2020 that have materially affected, or are reasonably likely to materially affect, our internal control over financial reporting.

PART II – OTHER INFORMATION

Item 1. Legal Proceedings

We are not currently a party to any material legal proceedings.

Item 1A. Risk Factors

In addition to the other information set forth in this Quarterly Report on Form 10-Q, you should carefully consider the factors discussed under the caption “Risk Factors” that appear in Item 1A of our Annual Report on Form 10-K for the year ended December 31, 2019, filed with the Securities and Exchange Commission (“SEC”) on March 2, 2020, as amended (the “2019 Annual Report on Form 10-K”), and in Item 1A of our Quarterly Report on Form 10-Q for the period ended March 31, 2020, which was filed with the SEC on May 11, 2020 (the “March 31 Quarterly Report on Form 10-Q”). There have been no material changes from the risk factors previously disclosed in the 2019 Annual Report on Form 10-K and in the March 31 Quarterly Report on Form 10-Q.

Item 2. Unregistered Sales of Equity Securities and Use of Proceeds

Recent Sales of Unregistered Equity Securities

None.

Issuer Purchases of Equity Securities

We did not repurchase any of our equity securities during the three months ended September 30, 2020.

Item 3. Defaults Upon Senior Securities

None.

Item 4. Mine Safety Disclosures

Not applicable.

Item 5. Other Information

None.

Item 6. Exhibits

<u>Exhibit Number</u>	<u>Exhibit Description</u>	<u>Filed with this Report</u>	<u>Incorporated by Reference herein from Form or Schedule</u>	<u>Filing Date</u>	<u>SEC File/Reg. Number</u>
10.1+	Non-Employee Director Compensation Policy, effective as of August 18, 2020	X			
31.1	Rule 13a-14(a)/15d-14(a) Certification of Principal Executive Officer	X			
31.2	Rule 13a-14(a)/15d-14(a) Certification of Principal Financial Officer	X			
32*	Section 1350 Certifications of Principal Executive Officer and Principal Financial Officer	X			
101.INS	Inline XBRL Instance Document – The instance document does not appear in the interactive data file because its Inline XBRL tags are embedded within the Inline XBRL document.	X			
101.SCH	Inline XBRL Taxonomy Extension Schema Document	X			
101.CAL	Inline XBRL Taxonomy Extension Calculation Linkbase Document	X			
101.DEF	Inline XBRL Taxonomy Extension Definition Linkbase Document	X			
101.LAB	Inline XBRL Taxonomy Extension Label Linkbase Document	X			
101.PRE	Inline XBRL Taxonomy Extension Presentation Linkbase Document	X			
104	Cover Page Interactive Data File (formatted in Inline XBRL and contained in Exhibit 101)	X			

(*) The certifications attached as Exhibit 32 that accompany this Quarterly Report on Form 10-Q are not deemed filed with the Securities and Exchange Commission and are not to be incorporated by reference into any filing of Wave Life Sciences Ltd. under the Securities Act of 1933, as amended, or the Securities Exchange Act of 1934, as amended (whether made before or after the date of such Form 10-Q), irrespective of any general incorporation language contained in such filing.

(+) Indicates management contract or compensatory plan or arrangement.

SIGNATURES

Pursuant to the requirements of the Securities Exchange Act of 1934, the Registrant has duly caused this report to be signed on its behalf by the undersigned thereunto duly authorized.

WAVE LIFE SCIENCES LTD.

Date: November 9, 2020

By: /s/ Paul B. Bolno, M.D., MBA
Paul B. Bolno, M.D., MBA
President and Chief Executive Officer
(Principal Executive Officer)

Date: November 9, 2020

By: /s/ David G. Gaiero
David G. Gaiero
Interim Chief Financial Officer (Principal Financial Officer and
Principal Accounting Officer)

Effective: August 18, 2020

**WAVE LIFE SCIENCES LTD.
NON-EMPLOYEE DIRECTOR COMPENSATION POLICY, AS AMENDED**

A. Introduction

Board of Directors (the “Board”) of Wave Life Sciences Ltd. (the “Company”) has approved the following Non-Employee Director Compensation Policy (this “Policy”), which establishes compensation to be paid to non-employee directors of the Company to provide an inducement to obtain and retain the services of qualified persons to serve as members of the Board.¹ This Policy shall be effective as of the date of receipt of the final voting results evidencing requisite shareholder approval of the non-employee director compensation proposal at the 2020 annual general meeting (the “Effective Time”) through the date of the Company’s 2021 annual general meeting, at which time the shareholders of the Company will be asked to approve the key parameters of a new or extended version of this Policy. Subject to receipt of shareholder approval, such new or extended policy shall take effect and that cycle will continue from annual general meeting to annual general meeting.

B. Applicable Persons

This Policy shall apply to each director of the Company who is not an employee of the Company or any Affiliate (each, an “Outside Director”). “Affiliate” shall mean a corporation which is a direct or indirect parent or subsidiary of the Company, as determined pursuant to Section 424 of the Internal Revenue Code of 1986, as amended.

C. Equity Compensation - Share Option Grants

The amounts set forth herein shall be subject to automatic adjustment in the event of any share split or other recapitalization affecting the Company’s ordinary shares (the “Ordinary Shares”) following the Effective Time.

(1) Initial Share Option Grants for Newly Appointed or Elected Directors

Each Outside Director appointed or elected on or after the Effective Time shall be granted a non-qualified share option to purchase 21,000 Ordinary Shares under the Company’s 2014 Equity Incentive Plan, as amended (the “2014 Plan”), on the date of his or her initial appointment or election to the Board (an “Initial Share Option Grant”). Initial Share Option Grants shall (i) vest as to 25% on the first anniversary of the grant date and vest as to the remaining 75% on a quarterly basis thereafter for the next three years, subject to the Outside Director’s continued service on the Board; provided that such options shall become exercisable in full immediately prior to and contingent upon the closing of a Change of Control of the Company (as defined in the option agreement); (ii) have an exercise price equal to the fair market value of the Ordinary Shares on the grant date; (iii) expire and no longer be exercisable after the five-year anniversary of the grant date; and (iv) contain such other terms and conditions as the Board or the Compensation Committee shall determine.

(2) Annual Share Option Grants

On the Effective Time, subject to receiving shareholders’ approval at the 2020 annual general meeting, each Outside Director (other than a new Outside Director who receives an Initial Share Option Grant) shall be

¹ This Policy, in its original form, was formulated and approved by the Board within the limits approved by the Company’s shareholders at the 2016 annual general meeting held on August 18, 2016. The Company first began compensating non-employee directors for their service on the Board on November 10, 2016.

granted a non-qualified share option to purchase 10,500 Ordinary Shares under the 2014 Plan (an “Annual Share Option Grant”). Annual Share Option Grants shall (i) vest as to 100% on the earlier of the 2021 annual general meeting or the first anniversary of the grant date, subject to the Outside Director’s continued service on the Board during that period; provided that such options shall become exercisable in full immediately prior to and contingent upon the closing of a Change of Control of the Company (as defined in the option agreement); (ii) have an exercise price equal to the fair market value of the Ordinary Shares on the grant date; (iii) expire and no longer be exercisable after the five-year anniversary of the grant date; and (iv) contain such other terms and conditions as the Board or the Compensation Committee shall determine.

D. Cash Compensation

(1) Annual Cash Fees

The following annual cash fees shall be paid to the Outside Directors serving on the Board and the Audit Committee, Compensation Committee and Nominating and Corporate Governance Committee, as applicable, provided that each non-employee director who receives cash fees as a committee member qualifies as an independent director pursuant to the definition promulgated by the Nasdaq Stock Market.

Board or Committee of Board	Annual Amount for Chair	Annual Amount for Member
Board	\$72,500	\$40,000
Audit Committee	\$16,000	\$8,000
Compensation Committee	\$12,000	\$6,000
Nominating and Corporate Governance Committee	\$10,000	\$5,000

(2) Payment Terms for All Cash Fees

As payable to Outside Directors shall be paid quarterly in arrears as of the last day of each fiscal quarter commencing on the later of the Effective Time or an Outside Director’s first election or appointment to the Board, prorated from the Effective Time or such Outside Director’s election or appointment date, as applicable. If an Outside Director dies, resigns or is removed during any quarter, he or she shall be entitled to a cash fee on a prorated basis through his or her last day of service.

E. Expenses

Upon presentation of documented expenses, reasonably satisfactory to the Company, each Outside Director shall be reimbursed for his or her reasonable, documented out-of-pocket business expenses incurred in connection with attending meetings of the Board and Committees thereof, or general meetings of shareholders, or in connection with other business related to the Board.

F. Amendments

The Compensation Committee or the Board shall review this Policy from time to time to assess whether any changes in the type or amount of compensation provided herein should be adjusted in order to fulfill the objectives of this Policy, provided, however, that changes to this Policy which require shareholder approval under applicable law shall require such shareholder approval to be obtained before taking effect.

CERTIFICATIONS UNDER SECTION 302

I, Paul B. Bolno, M.D., MBA, certify that:

1. I have reviewed this Quarterly Report on Form 10-Q of Wave Life Sciences Ltd.;
2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;
3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;
4. The registrant's other certifying officer(s) and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:
 - a) designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;
 - b) designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;
 - c) evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and
 - d) disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and
5. The registrant's other certifying officer(s) and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):
 - a) all significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and
 - b) any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

November 9, 2020

By: /s/ Paul B. Bolno, M.D., MBA
Paul B. Bolno, M.D., MBA
President and Chief Executive Officer
(Principal Executive Officer)

CERTIFICATIONS UNDER SECTION 302

I, David G. Gaiero, certify that:

1. I have reviewed this Quarterly Report on Form 10-Q of Wave Life Sciences Ltd.;
2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;
3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;
4. The registrant's other certifying officer(s) and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:
 - a) designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;
 - b) designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;
 - c) evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and
 - d) disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and
5. The registrant's other certifying officer(s) and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):
 - a) all significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and
 - b) any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

November 9, 2020

By: /s/ David G. Gaiero
David G. Gaiero
Interim Chief Financial Officer
(Principal Financial Officer)

CERTIFICATIONS UNDER SECTION 906

Pursuant to section 906 of the Sarbanes-Oxley Act of 2002 (subsections (a) and (b) of section 1350, chapter 63 of title 18, United States Code), each of the undersigned officers of Wave Life Sciences Ltd. (the “Company”), does hereby certify, to such officer’s knowledge, that:

The Quarterly Report for the quarter ended September 30, 2020 (the “Form 10-Q”) of the Company fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934, and the information contained in the Form 10-Q fairly presents, in all material respects, the financial condition and results of operations of the Company.

Dated: November 9, 2020

/s/ Paul B. Bolno, M.D., MBA

Paul B. Bolno, M.D., MBA
President and Chief Executive Officer
(Principal Executive Officer)

Dated: November 9, 2020

/s/ David G. Gaiero

David G. Gaiero
Interim Chief Financial Officer
(Principal Financial Officer)