

**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 March 31, 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 May 1, 2020 was 34,892,550.

**WAVE LIFE SCIENCES LTD.**  
**QUARTERLY REPORT ON FORM 10-Q**  
**TABLE OF CONTENTS**

	<u>Page</u>
<b><u>PART I - FINANCIAL INFORMATION</u></b>	5
<u>Item 1. Financial Statements</u>	5
<u>Unaudited Consolidated Balance Sheets</u>	5
<u>Unaudited Consolidated Statements of Operations and Comprehensive Loss</u>	6
<u>Unaudited Consolidated Statements of Series A Preferred Shares and Shareholders' Equity</u>	7
<u>Unaudited Consolidated Statements of Cash Flows</u>	8
<u>Notes to Unaudited Consolidated Financial Statements</u>	9
<u>Item 2. Management's Discussion and Analysis of Financial Condition and Results of Operations</u>	17
<u>Item 3. Quantitative and Qualitative Disclosures About Market Risk</u>	27
<u>Item 4. Controls and Procedures</u>	28
<b><u>PART II - OTHER INFORMATION</u></b>	28
<u>Item 1. Legal Proceedings</u>	28
<u>Item 1A. Risk Factors</u>	28
<u>Item 2. Unregistered Sales of Equity Securities and Use of Proceeds</u>	29
<u>Item 3. Defaults Upon Senior Securities</u>	29
<u>Item 4. Mine Safety Disclosures</u>	29
<u>Item 5. Other Information</u>	30
<u>Item 6. Exhibits</u>	31

### **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; and 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)

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

*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)*

	<b>Three Months Ended March 31,</b>	
	<b>2020</b>	<b>2019</b>
Revenue	\$ 4,161	\$ 3,026
Operating expenses:		
Research and development	41,158	40,113
General and administrative	12,996	10,901
Total operating expenses	54,154	51,014
Loss from operations	(49,993)	(47,988)
Other income, net:		
Dividend income	385	1,424
Interest income, net	3	11
Other income (expense), net	2,112	2,353
Total other income, net	2,500	3,788
Loss before income taxes	(47,493)	(44,200)
Income tax provision	—	—
Net loss	\$ (47,493)	\$ (44,200)
Net loss per share attributable to ordinary shareholders—basic and diluted	\$ (1.38)	\$ (1.36)
Weighted-average ordinary shares used in computing net loss per share attributable to ordinary shareholders—basic and diluted	34,461,505	32,597,158
Other comprehensive income (loss):		
Net loss	\$ (47,493)	\$ (44,200)
Foreign currency translation	6	97
Comprehensive loss	\$ (47,487)	\$ (44,103)

*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				
<b>Balance at December 31, 2018</b>	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)
<b>Balance at March 31, 2019</b>	<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>

	Series A Preferred Shares		Ordinary Shares		Additional Paid-In- Capital	Accumulated Other Comprehensive Income	Accumulated Deficit	Total Shareholders' Equity
	Shares	Amount	Shares	Amount				
<b>Balance at December 31, 2019</b>	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)
<b>Balance at March 31, 2020</b>	<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>

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

**WAVE LIFE SCIENCES LTD.**  
**UNAUDITED CONSOLIDATED STATEMENTS OF CASH FLOWS**

(In thousands)

	Three Months Ended March 31,	
	2020	2019
<b>Cash flows from operating activities</b>		
Net loss	\$ (47,493)	\$ (44,200)
Adjustments to reconcile net loss to net cash used in operating activities:		
Amortization of right-of-use assets	442	381
Depreciation of property and equipment	2,039	1,783
Share-based compensation expense	3,999	4,345
Changes in operating assets and liabilities:		
Accounts receivable	20,000	—
Prepaid expenses	(373)	(1,090)
Other assets	(1,983)	(2,124)
Accounts payable	7,336	783
Accrued expenses and other current liabilities	(5,191)	(6,467)
Deferred revenue	(4,161)	(3,026)
Operating lease liabilities	(765)	(663)
Other non-current liabilities	(100)	(103)
Net cash used in operating activities	(26,250)	(50,381)
<b>Cash flows from investing activities</b>		
Purchases of property and equipment	(580)	(508)
Net cash used in investing activities	(580)	(508)
<b>Cash flows from financing activities</b>		
Proceeds from issuance of ordinary shares, net of offering costs	—	162,065
Proceeds from issuance of ordinary shares pursuant to the at-the-market equity program, net	604	—
Proceeds from the exercise of share options	10	1,481
Net cash provided by financing activities	614	163,546
Effect of foreign exchange rates on cash, cash equivalents and restricted cash	6	97
Net increase (decrease) in cash, cash equivalents and restricted cash	(26,210)	112,754
Cash, cash equivalents and restricted cash, beginning of period	150,808	178,444
Cash, cash equivalents and restricted cash, end of period	\$ 124,598	\$ 291,198
<b>Supplemental disclosure of cash flow information:</b>		
Deferred follow-on offering costs in accounts payable and accrued expenses at period end	\$ —	\$ 280

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

## Notes to 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 March 31, 2020, the Company had cash and cash equivalents of \$120.9 million. The Company expects that its existing cash and cash equivalents 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 to remain focused 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 ultimate geographic spread 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 months ended March 31, 2020.

### ***Unaudited Interim Financial Data***

The accompanying interim consolidated balance sheet as of March 31, 2020, the related interim consolidated statements of operations and comprehensive loss for the three months ended March 31, 2020 and 2019, the consolidated statements of Series A preferred shares and shareholders' equity for the three months ended March 31, 2020 and 2019, the consolidated statements of cash flows for the three months ended March 31, 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 months ended March 31, 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 months ended March 31, 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 three months ended March 31, 2020.

### 3. OTHER CURRENT ASSETS

Other current assets consist of the following:

	March 31, 2020	December 31, 2019
	(in thousands)	
Refundable tax credits receivable	\$ 18,692	\$ 8,205
Dividend income receivable	104	200
Other current assets	47	284
Total other current assets	<u>\$ 18,843</u>	<u>\$ 8,689</u>

### 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 three months ended March 31, 2020, the Company granted 701,685 options and 14,750 RSUs to employees.

As of March 31, 2020, 878,110 ordinary shares remained available for future grant under the 2014 Plan.

#### *Employee Share Purchase Plan*

At the 2019 Annual General Meeting, the Company’s board of directors and the Company’s shareholders approved the 2019 Employee Share Purchase Plan (“ESPP”). As of March 31, 2020, there were 1,000,000 ordinary shares available for issuance under the ESPP. The 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 approximately every January 15<sup>th</sup> and July 15<sup>th</sup>. 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 three months ended March 31, 2020, no shares were issued under the ESPP and the earliest that shares would be issued under the ESPP is July 14, 2020.

### 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 may earn potential 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. During the research term, the Company is responsible to use its commercially reasonable efforts to advance up to five programs through to the selection of clinical candidates. At that stage, Pfizer may 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 uses this technology on the Wave Programs, Pfizer is eligible to receive potential development and commercial milestone payments from the Company. Pfizer is also eligible to receive tiered royalties on sales of any products that include Pfizer's hepatic targeting technology. The Company is not currently utilizing 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 terminates on the date of the last to expire payment obligation with respect to each Pfizer Program and, with respect to each Wave Program, expires on a program-by-program basis accordingly. Pfizer may 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 may also terminate its rights related to a Wave Program at its own convenience upon 90 days' notice to Pfizer. The Pfizer Collaboration Agreement may 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 provides 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 is managed by a joint steering committee in which both parties are represented equally, which will oversee 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 has agreed to work exclusively with Pfizer with respect to using any of the Company's stereopure oligonucleotide technology that is 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 may 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, is 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 are subject to Pfizer's exercise of the program nomination options for such programs and therefore do 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 do not represent material rights; as such, they are 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 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 Pfizer Collaboration Agreement. The exercise fees for the options to obtain a license to develop, manufacture and commercialize Programs 3, 4 and 5 that may be received are excluded from the transaction price until each customer option is exercised. 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, and, if necessary, will adjust 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 will be recognized in the same manner as the remaining, unrecognized transaction price over the remaining period until each performance obligation is satisfied.

Revenue associated with the performance obligations relating to Programs 1 and 2 is being recognized as revenue as the research and development services are 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 occurs over time and, in management’s judgment, this input method is the best measure of progress towards satisfying the performance obligation. The amount allocated to the three material rights will be recognized as the underlying research and development services are provided commencing from the date that Pfizer 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.

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 will be recognized as revenue as the research and development services are provided using the same method as the performance obligations relating to Programs 1 and 2.

Through March 31, 2020, the Company had recognized revenue of \$18.3 million as collaboration revenue in the Company’s consolidated statements of operations and comprehensive loss under the Pfizer Collaboration Agreement. During the three months ended March 31, 2020 and 2019, the Company recognized revenue of approximately \$1.3 million and \$0.6 million, respectively, in the Company’s consolidated statements of operations and comprehensive loss under the Pfizer Collaboration Agreement. The aggregate amount of the transaction price allocated to the Company’s partially unsatisfied performance obligations and recorded in deferred revenue at March 31, 2020 is \$0.2 million, all of which is included in current liabilities. The Company expects to recognize this amount according to FTE hours incurred, over the remaining research term, which is one month as of March 31, 2020.

### ***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 March 31, 2020, the Company had recognized revenue of approximately \$21.3 million as collaboration revenue in the Company's consolidated statements of operations and comprehensive loss under the Takeda Collaboration Agreement. During the three months ended March 31, 2020 and 2019, the Company recognized revenue of \$2.9 million and \$2.5 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 March 31, 2020 is \$148.7 million, of which \$87.8 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 March 31, 2020 is \$30.0 million, all of which is included in long-term assets.

## **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:

	<u>As of March 31,</u>	
	<u>2020</u>	<u>2019</u>
Options to purchase ordinary shares	4,320,605	3,853,542
RSUs and PSUs	1,272,350	1,674,124
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 months ended March 31, 2020 and 2019, the Company recorded no income tax provision.

The Company maintained a full valuation allowance for the three months ended March 31, 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 March 31, 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, which has not otherwise been discussed in these notes to the 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") and to cease development of the Company's other DMD programs. 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 three months ended March 31, 2020.

During the three months ended March 31, 2020, the Company paid approximately \$2.1 million of these restructuring expenses. The remaining payments of \$1.3 million will be paid through March 31, 2021.

	<u>Accrued Restructuring Expenses at January 1, 2020</u>	<u>Expenses</u>	<u>Less: Payments</u>	<u>Accrued Restructuring Expenses at March 31, 2020</u>
	(in thousands)			
Severance, benefits and related costs due to workforce reduction	\$ —	\$ 3,385	\$ (2,096)	\$ 1,289
Total	<u>\$ —</u>	<u>\$ 3,385</u>	<u>\$ (2,096)</u>	<u>\$ 1,289</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 to remain focused 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 of COVID-19 on our business, which continue to evolve with the rapid evolution of the pandemic. 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. Our COVID-19 Response Team continues to assess, evaluate and evolve our ongoing business continuity plans and our COVID-19 policies and guidance.

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.

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 neurology programs approaching clinical development include our mHTT SNP3 program for the treatment of HD and our C9orf72 program for the treatment of amyotrophic lateral sclerosis (“ALS”) and frontotemporal dementia (“FTD”). We are also pursuing additional CNS programs in collaboration with Takeda Pharmaceutical Company Limited (“Takeda”), including spinocerebellar ataxia 3 (“SCA3”). Outside of neurology, we are advancing discovery research in ophthalmologic disorders, specifically inherited retinal diseases, in hepatic diseases, and in ADAR-mediated RNA-editing applications. 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.

## Our Current Programs

THERAPEUTIC AREA	TARGET	DISCOVERY	PRECLINICAL	CLINICAL	ESTIMATED U.S. PREVALENCE*	PARTNER
<b>NEUROLOGY</b>						
Huntington's disease	WVE-120101 mHTT SNP1	Phase 1b/2a and OLE			~10,000 / ~35,000	Takeda 50:50 option
	WVE-120102 mHTT SNP2	Phase 1b/2a and OLE			~10,000 / ~35,000	Takeda 50:50 option
	mHTT SNP3				~8,000 / ~30,000	Takeda 50:50 option
ALS and FTD	C9orf72				~1,800 (ALS) ~7,000 (FTD)	Takeda 50:50 option
Spinocerebellar ataxia 3	ATXN3				~4,500	Takeda 50:50 option
CNS diseases	Multiple†					Takeda milestones & royalties
<b>OPHTHALMOLOGY</b>						
Retinal diseases	USH2A and RhoP23H					100% global
<b>HEPATIC</b>						
ADAR RNA-editing	Multiple					100% global

\*Estimates of U.S. prevalence and addressable population by target based on publicly available data and are approximate; for Huntington's disease, numbers approximate manifest and pre-manifest populations, respectively.  
†During a four-year term, Wave and Takeda may collaborate on up to six preclinical targets at any one time.  
ALS: Amyotrophic lateral sclerosis; FTD: Frontotemporal dementia; CNS: Central nervous system; OLE: Open-label extension

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, 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. Our mHTT SNP3 program is currently in the preclinical stage. 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, our SNP3 compounds selectively reduce the expression of the mutant HTT.

**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. If global restrictions continue or worsen, the ability to evaluate patients in both of the PRECISION-HD trials as planned may be 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 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 in the second half of 2020.

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

**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.

**mHTT SNP3 Program in HD:** We expect to initiate clinical development of our mHTT SNP3 program in the second half of 2020.

**ALS and FTD:** In amyotrophic lateral sclerosis ("ALS") and frontotemporal dementia ("FTD"), we are advancing our C9orf72 program, which is designed to selectively target the transcripts containing the hexanucleotide repeat expansion (G4C2) in the C9orf72 gene. Our C9orf72 program 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 our C9orf72 program in the second half 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.

### **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.

## *Hepatic*

In May 2020, we announced the first *in vivo* data from our novel RNA-editing platform, which demonstrated successful RNA editing of ACTB (Beta-actin) mRNA in NHPs via endogenous ADARs using stereopure GalNAc-conjugated oligonucleotides. In this ongoing proof-of-concept study, our oligonucleotides demonstrated up to 50% A to I (G) editing of ACTB mRNA in the liver of NHPs two-days post-last dose. To our knowledge, these are the first publicly available data that demonstrate successful RNA editing *in vivo* in NHPs.

We recently concluded our collaboration with Pfizer Inc. to advance genetically defined targets for the treatment of metabolic diseases. Through our work in the collaboration, we have incorporated valuable learnings and technology advancements into PRISM, where we have leveraged our new understanding of GalNAc conjugations to design novel RNA-editing oligonucleotides.

## **Recent Developments**

On February 6, 2020, we implemented a plan to reduce operating costs and better align our workforce with the needs of our business following our December 16, 2019 announcement of our decision to discontinue the suvodirsen program for patients with DMD and to cease development of our other DMD programs. Under this cost reduction plan, we reduced our workforce by approximately 22%. The reduction in workforce was completed in February 2020.

## **Financial Operations Overview**

We have never been profitable, and since our inception, we have incurred significant operating losses. Our net loss was \$47.5 million and \$44.2 million in the three months ended March 31, 2020 and 2019, respectively. As of March 31, 2020 and December 31, 2019, we had an accumulated deficit of \$580.9 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 three months ended March 31, 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 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 months ended March 31, 2020 and 2019:

	<b>Three Months Ended March 31,</b>	
	<b>2020</b>	<b>2019</b>
	(in thousands)	
DMD programs	\$ 4,509	\$ 12,448
HD programs	7,513	3,438
ALS and FTD programs	2,038	726
PRISM and other research and development expenses (1)	27,098	23,501
<b>Total research and development expenses</b>	<b>\$ 41,158</b>	<b>\$ 40,113</b>

- (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, Net**

Other income, net consists primarily of 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 March 31, 2020 and 2019

	Three Months Ended March 31,		Change
	2020	2019	
	(in thousands)		
Revenue	\$ 4,161	\$ 3,026	\$ 1,135
Operating expenses:			
Research and development	41,158	40,113	1,045
General and administrative	12,996	10,901	2,095
Total operating expenses	54,154	51,014	3,140
Loss from operations	(49,993)	(47,988)	(2,005)
Other income, net:	2,500	3,788	(1,288)
Loss before income taxes	(47,493)	(44,200)	(3,293)
Income tax provision	—	—	—
Net loss	\$ (47,493)	\$ (44,200)	\$ (3,293)

### Revenue

Revenue of approximately \$4.2 million and \$3.0 million was earned under the Pfizer Collaboration Agreement and the Takeda Collaboration Agreement for the three months ended March 31, 2020 and 2019, respectively. The \$1.1 million increase in revenue is due to an increase in research and development services under the Pfizer Collaboration Agreement and the Takeda Collaboration Agreement.

### Research and Development Expenses

	Three Months Ended March 31,		Change
	2020	2019	
	(in thousands)		
DMD programs	\$ 4,509	\$ 12,448	\$ (7,939)
HD programs	7,513	3,438	4,075
ALS and FTD programs	2,038	726	1,312
PRISM and other research and development expenses (1)	27,098	23,501	3,597
Total research and development expenses	\$ 41,158	\$ 40,113	\$ 1,045

- (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 \$41.2 million for the three months ended March 31, 2020, compared to \$40.1 million for the three months ended March 31, 2019. The increase of approximately \$1.0 million was due to the following:

- an increase of \$4.1 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 \$1.3 million in external expenses related to our ALS and FTD programs;
- an increase of \$3.6 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 increases in compensation-related expenses, including employee severance, benefits and related termination costs due to the February 2020 reduction in workforce; and
- a decrease of \$7.9 million in external expenses related to our DMD programs, including suvodirsén, mainly due to our December 2019 decision to discontinue the suvodirsén program and to cease development of our other DMD programs.

#### *General and Administrative Expenses*

General and administrative expenses were \$13.0 million for the three months ended March 31, 2020, as compared to \$10.9 million for the three months ended March 31, 2019. The increase of \$2.1 million was mainly driven by compensation-related costs, including employee severance, benefits and related termination costs due to the February 2020 reduction in workforce.

#### *Other Income, Net*

Other income, net for the three months ended March 31, 2020 and 2019 was \$2.5 million and \$3.8 million, respectively. The decrease of \$1.3 million in other income, net is primarily due to decreased dividend income earned on cash and cash equivalents.

#### *Income Tax Provision*

During the three months ended March 31, 2020 and 2019, we recorded no income tax provision. We maintained a full valuation allowance for the three months ended March 31, 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 March 31, 2020, we have received an aggregate of approximately \$655.0 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, and \$161.8 million in net proceeds from our January 2019 follow-on underwritten public offering.

As of March 31, 2020, we had cash and cash equivalents totaling \$120.9 million, an accumulated deficit of \$580.9 million and restricted cash of \$3.6 million for our leased premises in Cambridge, Massachusetts and Lexington, Massachusetts.

We expect that our existing cash and cash equivalents 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 three months ended March 31, 2020, we sold approximately 60,000 ordinary shares under our at-the market equity program for aggregate net proceeds of approximately \$0.6 million. As of this filing on May 11, 2020, we have sold an aggregate of approximately 357,000 ordinary shares resulting in aggregate net proceeds of approximately \$3.4 million under our at-the-market equity program, thereby leaving approximately \$246.6 million in ordinary shares available for sale thereunder.

Adequate additional financing may not be available to us on acceptable terms, or at all. Raising 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:

	<b>Three Months Ended March 31,</b>	
	<b>2020</b>	<b>2019</b>
	(in thousands)	
Net cash used in operating activities	\$ (26,250)	\$ (50,381)
Net cash used in investing activities	(580)	(508)
Net cash provided by financing activities	614	163,546
Effect of foreign exchange rates on cash, cash equivalents and restricted cash	6	97
Net increase (decrease) in cash, cash equivalents and restricted cash	<u>\$ (26,210)</u>	<u>\$ 112,754</u>

### *Operating Activities*

During the three months ended March 31, 2020, operating activities used \$26.3 million of cash, primarily due to our net loss of \$47.5 million, offset by a \$20.0 million decrease in accounts receivable.

During the three months ended March 31, 2019, operating activities used approximately \$50.4 million of cash, primarily due to our net loss of \$44.2 million and changes in our operating assets and liabilities of \$12.7 million, offset by non-cash charges of \$6.5 million. The largest changes in operating assets and liabilities were a decrease of \$6.5 million in accrued expenses and other current liabilities, as well as a decrease of \$3.0 million in deferred revenue. The non-cash charges for the three months ended March 31, 2019 were mainly related to share-based compensation expense of \$4.3 million and depreciation expense of \$1.8 million.

### *Investing Activities*

During the three months ended March 31, 2020, investing activities used \$0.6 million of cash, related to purchases of property and equipment.

During the three months ended March 31, 2019, investing activities used \$0.5 million of cash, related to purchases of property and equipment.

### *Financing Activities*

During the three months ended March 31, 2020, net cash provided by financing activities was \$0.6 million, which was primarily due to the net proceeds from sales of ordinary shares under our at-the-market equity program.

During the three months ended March 31, 2019, net cash provided by financing activities was \$163.6 million, which was primarily due to the \$162.1 million in net proceeds from our underwritten public offering of ordinary shares in January 2019 and approximately \$1.5 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 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 March 31, 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 three months ended March 31, 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 months ended March 31, 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 months ended March 31, 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 March 31, 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 March 31, 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 March 31, 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 below and 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 on March 2, 2020, as amended.

***We, or third parties upon whom we depend, may face risks related to health epidemics, including the novel coronavirus (COVID-19) pandemic, which may delay our ability to complete our ongoing clinical trials, initiate additional clinical trials, delay regulatory activities and have other adverse effects on our business and operations.***

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, including the United States, 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, including the United States. We are in the midst of the global pandemic, therefore the extent to which these responsive measures may materially and adversely affect our business operations and financial condition is currently unclear and we are continuing to evaluate the situation.

As a clinical-stage company with multiple programs and multiple clinical trials currently underway, the pandemic is impacting the execution of our clinical trials. We have clinical trial sites located in countries that have been affected by COVID-19. Clinical site initiation and patient enrollment may be delayed due to prioritization of hospital resources in favor of COVID-19 patients and difficulties in recruiting clinical site investigators and clinical site staff. Some patients may not be able to comply with clinical trial protocols if quarantines impede patient movement or interrupt healthcare services, or patients may not be willing to travel to clinical trial sites. Similarly, our ability to recruit and retain patients and principal investigators and site staff who, as healthcare providers, may have heightened exposure to COVID-19 and adversely impact our clinical trial operations. We may also experience delays or disruptions in our clinical trials or preclinical studies due to unforeseen circumstances at contract research organizations and vendors along their supply chain. We may also experience interruption of key clinical trial activities, such as clinical trial site data monitoring, due to limitations on travel imposed or recommended by federal or state governments, employers and others or interruption of clinical trial subject visits and study procedures (particularly any procedures that may be deemed non-essential), which may impact the integrity of subject data and clinical trial endpoints.

The COVID-19 pandemic has affected and may continue to affect the operations of the FDA, EMA and other regulatory authorities, which could result in delays of reviews and approvals, including with respect to our product candidates. If regulatory matters resulting from COVID-19 continue to prevent regulatory authorities from conducting their regular inspections, reviews, or other regulatory activities, it could impact the ability of regulatory authorities to timely review and process our regulatory submissions, which could have a material adverse effect on our business.

We rely upon third parties for many aspects of our business, including the raw materials used to make our product candidates and the conduct of our clinical trials and preclinical studies. While we have built up inventory to assist us through this uncertain operating environment, our suppliers may be disrupted now or in the future, which may affect our ability to procure items that are essential for our research and development activities and may cause significant disruptions to our business.

We have implemented procedures to protect our workforce in manufacturing and laboratory operations while ensuring appropriate remote working protocols have been implemented for our employees who can work from home. The spread of COVID-19 and the responsive measures taken to date have limited our access to our facilities and caused the majority of our employees to work from home. We continue to monitor the global spread of COVID-19 and the response of international, national and local authorities, and have implemented and will continue to implement measures that we believe are appropriate and necessary for our business and the safety of our employees. In response to these public health directives and orders, we have implemented a work-from-home policy for our employees. The effects of the executive order, the stay-at-home advisory and our work-from-home policy may negatively impact productivity, disrupt our business and delay our clinical programs and timelines, the magnitude of which will depend, in part, on the length and severity of the restrictions and other limitations on our ability to conduct our business in the ordinary course. The increase in working remotely may increase our cybersecurity risk, create data accessibility concerns, and make us more susceptible to communication disruptions. In addition, as a result of potential shelter-in-place orders or other mandated travel restrictions, our on-site staff conducting research and development and manufacturing activities may experience difficulties or delays in accessing our laboratories or manufacturing space.

Our future capital requirements depend on many factors, including the uncertain impact of the COVID-19 pandemic. The pandemic has already caused significant disruptions to the financial markets, and may continue to cause such disruptions, which could impact our ability to raise capital. Raising 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. As a result, we may face difficulties raising capital through sales of our securities or such sales may be on unfavorable terms.

The COVID-19 global pandemic is evolving rapidly and its impact on us is highly uncertain and subject to change. We do not yet know the full extent of potential delays or long-term impacts on our business, our clinical trials, healthcare systems or the global economy. These impacts are highly uncertain and cannot be predicted with confidence, such as the ultimate geographic spread 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 in the United States and other countries, business closures or business disruptions and the effectiveness of actions taken in the United States and other countries to contain and treat the disease. These effects may materially adversely affect our business, financial condition, results of operations, and prospects.

## **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 March 31, 2020.

## **Item 3. Defaults Upon Senior Securities**

None.

## **Item 4. Mine Safety Disclosures**

Not applicable.

## Item 5. Other Information

The information set forth in this Item 5 is included herein for the purpose of providing the disclosure required under “Item 5.02 - Departure of Directors or Certain Officers; Election of Directors; Appointment of Certain Officers; Compensatory Arrangements of Certain Officers” of Form 8-K.

On May 8, 2020, the Company entered into amended and restated employment agreements (the “Employment Agreements”) with certain of its named executive officers (each, an “Executive”): Paul B. Bolno, M.D., MBA; and Chandra Vargeese, Ph.D. The Employment Agreements amend and restate the prior employment arrangements between the Company and such Executives. Under the terms of the Employment Agreements, the 2020 annual base salaries for Drs. Bolno and Vargeese are \$578,977 and \$434,520, respectively, and their annual target bonus percentages are 65% and 40% of their base salary, respectively.

The terms of the Employment Agreements provide that, if Drs. Bolno or Vargeese is involuntarily terminated by the Company without cause or such Executive terminates his or her employment for good reason, such Executive will be entitled to receive continued payment of his or her base salary for 18 months or 12 months, respectively, following termination; continued payment of health insurance premiums at the Company’s then normal rate of contribution until the earlier of 18 months or 12 months, respectively, following termination or until he or she commences new employment; and the payment of a separation bonus equal to his or her then annual target bonus opportunity, prorated through the termination date.

In addition, if a change of control occurs and within one year following the change of control Drs. Bolno or Vargeese is involuntarily terminated without cause or such Executive terminates his or her employment for good reason, such Executive will be entitled to receive a lump sum cash payment equal to 18 months or 12 months, respectively, of his or her then-current annual base salary; continued payment of health insurance premiums at the Company’s then normal rate of contribution until the earlier of 18 months or 12 months, respectively, following termination or until he or she commences new employment; and the payment of a separation bonus equal to his or her then annual target bonus opportunity.

Receipt of the severance and change of control benefits described above are subject to execution of a release of claims against the Company and compliance with certain restrictive covenants following the termination of his or her employment.

Copies of the Employment Agreements will be filed as exhibits to our Quarterly Report on Form 10-Q for the quarter ending June 30, 2020. The foregoing descriptions of the terms of the Employment Agreements are qualified in their entirety by reference to the full text of such exhibits.

**Item 6. Exhibits**

<b>Exhibit Number</b>	<b>Exhibit Description</b>	<b>Filed with this Report</b>	<b>Incorporated by Reference herein from Form or Schedule</b>	<b>Filing Date</b>	<b>SEC File/Reg. Number</b>
10.1	<a href="#">Amendment No. 1 to Open Market Sale Agreement, dated as of March 2, 2020, by and between the Registrant and Jefferies LLC</a>		POSASR (Exhibit 1.3)	03/02/2020	333-231382
31.1	<a href="#">Rule 13a-14(a)/15d-14(a) Certification of Principal Executive Officer</a>	X			
31.2	<a href="#">Rule 13a-14(a)/15d-14(a) Certification of Principal Financial Officer</a>	X			
32*	<a href="#">Section 1350 Certifications of Principal Executive Officer and Principal Financial Officer</a>	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.

## 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: May 11, 2020

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

Date: May 11, 2020

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

## 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.

May 11, 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.

May 11, 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 March 31, 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: May 11, 2020

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

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

Dated: May 11, 2020

/s/ David G. Gaiero

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