

**UNITED STATES  
SECURITIES AND EXCHANGE COMMISSION  
Washington, D.C. 20549**

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**FORM 10-Q**

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(Mark One)

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

For the quarterly period ended June 30, 2019

OR

**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 001-37553

**REGENXBIO Inc.**

(Exact Name of Registrant as Specified in its Charter)

**Delaware**  
(State or other jurisdiction of  
incorporation or organization)

**9600 Blackwell Road, Suite 210**  
**Rockville, MD**  
(Address of principal executive offices)

**47-1851754**  
(I.R.S. Employer  
Identification No.)

**20850**  
(Zip Code)

**(240) 552-8181**

(Registrant's telephone number, including area code)

**Not Applicable**

(Former name, former address and former fiscal year, if changed since last report)

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

| Title of each class                        | Ticker symbol(s) | Name of each exchange on which registered |
|--|------------------|---|
| Common Stock, \$0.0001 par value per share | RGNX             | The Nasdaq Global Select 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

As of August 2, 2019, there were 36,806,632 outstanding shares of the registrant's common stock, par value \$0.0001 per share.

**REGENXBIO INC.**  
**QUARTERLY REPORT ON FORM 10-Q**  
**FOR THE QUARTERLY PERIOD ENDED JUNE 30, 2019**

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## INFORMATION 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 (the Securities Act), and Section 21E of the Securities Exchange Act of 1934, as amended (the Exchange Act). These statements express a belief, expectation or intention and are generally accompanied by words that convey projected future events or outcomes such as “believe,” “may,” “will,” “estimate,” “continue,” “anticipate,” “assume,” “design,” “intend,” “expect,” “could,” “plan,” “potential,” “predict,” “seek,” “should,” “would” or by variations of such words or by similar expressions. We have based these forward-looking statements on our current expectations and assumptions and analyses made by us in light of our experience and our perception of historical trends, current conditions and expected future developments, as well as other factors we believe are appropriate under the circumstances. However, whether actual results and developments will conform with our expectations and predictions is subject to a number of risks, uncertainties, assumptions and other important factors, including, but not limited to:

- the timing of enrollment, commencement and completion and the success of our clinical trials;
- the timing of commencement and completion and the success of preclinical studies conducted by us and our development partners;
- the timely development and launch of new products;
- the ability to obtain and maintain regulatory approval of our product candidates, and the labeling for any approved products;
- the scope, progress, expansion and costs of developing and commercializing our product candidates;
- our ability to obtain and maintain intellectual property protection for our product candidates and technology;
- our anticipated growth strategies;
- our expectations regarding competition;
- the anticipated trends and challenges in our business and the market in which we operate;
- our ability to attract or retain key personnel;
- the size and growth of the potential markets for our product candidates and the ability to serve those markets;
- the rate and degree of market acceptance of any of our product candidates;
- our ability to establish and maintain development partnerships;
- our expectations regarding our expenses and revenue;
- our expectations regarding regulatory developments in the United States and foreign countries; and
- the use or sufficiency of our cash and cash equivalents and needs for additional financing.

You should carefully read the factors discussed in the sections titled “Risk Factors,” “Management’s Discussion and Analysis of Financial Condition and Results of Operations” and elsewhere in this Quarterly Report on Form 10-Q, our Annual Report on Form 10-K for the year ended December 31, 2018 and in our other filings with the U.S. Securities and Exchange Commission (the SEC) for additional discussion of the risks, uncertainties, assumptions and other important factors that could cause our actual results or developments to differ materially and adversely from those projected in the forward-looking statements. The actual results or developments anticipated may not be realized or, even if substantially realized, they may not have the expected consequences to or effects on us or our businesses or operations. Such statements are not guarantees of future performance and actual results or developments may differ materially and adversely from those projected in the forward-looking statements. These forward-looking statements speak only as of the date of this report. Except as required by law, we disclaim any duty to update any forward-looking statements, whether as a result of new information, future events or otherwise.

## **Available Information**

We file annual, quarterly, and current reports, proxy statements, and other documents with the SEC under the Exchange Act. You may obtain any reports, proxy and information statements, and other information that we file electronically with the SEC at [www.sec.gov](http://www.sec.gov).

You also may view and download copies of our SEC filings free of charge at our website, [www.regenxbio.com](http://www.regenxbio.com), as soon as reasonably practicable after we electronically file such material with, or furnish it to, the SEC. The information contained on, or that can be accessed through, our website will not be deemed to be incorporated by reference in, and is not considered part of, this Quarterly Report on Form 10-Q. Investors should also note that we use our website, as well as SEC filings, press releases, public conference calls and webcasts, to announce financial information and other material developments regarding our business. We use these channels, as well as any social media channels listed on our website, to communicate with investors and members of the public about our business. It is possible that the information that we post on our social media channels could be deemed material information. Therefore, we encourage investors, the media and others interested in our company to review the information that we post on our social media channels.

As used in this Quarterly Report on Form 10-Q, the terms “REGENXBIO,” “we,” “us,” “our” or the “Company” mean REGENXBIO Inc. and its subsidiaries, on a consolidated basis, unless the context indicates otherwise.

NAV, REGENXBIO and the REGENXBIO logos are our registered trademarks. Any other trademarks appearing in this Quarterly Report on Form 10-Q are the property of their respective holders.

## PART I – FINANCIAL INFORMATION

## Item 1. Financial Statements.

**REGENXBIO INC.**  
**CONSOLIDATED BALANCE SHEETS**  
(unaudited)  
(in thousands, except per share data)

|  | June 30, 2019     | December 31, 2018 |
|--|-------------------|-------------------|
| <b>Assets</b>  |                   |                   |
| Current assets   |                   |                   |
| Cash and cash equivalents  | \$ 55,142         | \$ 75,561         |
| Marketable securities  | 286,354           | 244,200           |
| Accounts receivable  | 9,679             | 8,587             |
| Prepaid expenses   | 6,036             | 5,734             |
| Other current assets   | 2,281             | 3,831             |
| Total current assets   | 359,492           | 337,913           |
| Marketable securities  | 108,194           | 150,819           |
| Accounts receivable  | 23,955            | 23,012            |
| Property and equipment, net  | 27,330            | 28,702            |
| Operating lease right-of-use assets  | 5,904             | —                 |
| Restricted cash  | 1,053             | 1,053             |
| Other assets   | 3,211             | 2,315             |
| Total assets   | <u>\$ 529,139</u> | <u>\$ 543,814</u> |
| <b>Liabilities and Stockholders' Equity</b>  |                   |                   |
| Current liabilities  |                   |                   |
| Accounts payable   | \$ 5,851          | \$ 4,412          |
| Accrued expenses and other current liabilities   | 16,534            | 17,164            |
| Deferred revenue   | —                 | 600               |
| Operating lease liabilities  | 2,276             | —                 |
| Total current liabilities  | 24,661            | 22,176            |
| Deferred revenue   | 3,333             | 3,333             |
| Operating lease liabilities  | 4,654             | —                 |
| Deferred rent  | —                 | 1,098             |
| Financing lease obligations  | —                 | 5,854             |
| Other liabilities  | 1,899             | 2,505             |
| Total liabilities  | 34,547            | 34,966            |
| Commitments and contingencies (Note 7)   |                   |                   |
| Stockholders' equity   |                   |                   |
| Preferred stock; \$0.0001 par value; 10,000 shares authorized, and no shares issued and outstanding at June 30, 2019 and December 31, 2018   | —                 | —                 |
| Common stock; \$0.0001 par value; 100,000 shares authorized at June 30, 2019 and December 31, 2018; 36,752 and 36,120 shares issued and outstanding at June 30, 2019 and December 31, 2018, respectively | 4                 | 4                 |
| Additional paid-in capital   | 610,891           | 592,580           |
| Accumulated other comprehensive income (loss)  | 471               | (720)             |
| Accumulated deficit  | (116,774)         | (83,016)          |
| Total stockholders' equity   | 494,592           | 508,848           |
| Total liabilities and stockholders' equity   | <u>\$ 529,139</u> | <u>\$ 543,814</u> |

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

**REGENXBIO INC.**  
**CONSOLIDATED STATEMENTS OF OPERATIONS AND COMPREHENSIVE INCOME (LOSS)**  
**(unaudited)**  
**(in thousands, except per share data)**

|   | <u>Three Months Ended June 30,</u> |             | <u>Six Months Ended June 30,</u> |             |
|---|------------------------------------|-------------|----------------------------------|-------------|
|   | <u>2019</u>                        | <u>2018</u> | <u>2019</u>                      | <u>2018</u> |
| <b>Revenues</b>   |                                    |             |                                  |             |
| License and royalty revenue   | \$ 7,881                           | \$ 40,031   | \$ 8,765                         | \$ 172,422  |
| Total revenues  | 7,881                              | 40,031      | 8,765                            | 172,422     |
| <b>Operating Expenses</b>   |                                    |             |                                  |             |
| Cost of revenues  | 1,927                              | 3,872       | 1,956                            | 6,280       |
| Research and development  | 29,483                             | 21,486      | 54,686                           | 41,036      |
| General and administrative  | 13,405                             | 8,318       | 24,963                           | 16,698      |
| Other operating expenses (income)   | (62)                               | 5           | (62)                             | 33          |
| Total operating expenses  | 44,753                             | 33,681      | 81,543                           | 64,047      |
| Income (loss) from operations   | (36,872)                           | 6,350       | (72,778)                         | 108,375     |
| <b>Other Income</b>   |                                    |             |                                  |             |
| Interest income from licensing  | 762                                | 6,898       | 1,375                            | 8,253       |
| Investment income   | 34,524                             | 1,196       | 37,519                           | 2,055       |
| Total other income  | 35,286                             | 8,094       | 38,894                           | 10,308      |
| Income (loss) before income taxes   | (1,586)                            | 14,444      | (33,884)                         | 118,683     |
| <b>Income Tax Benefit (Expense)</b>   |                                    |             |                                  |             |
| Net income (loss)   | \$ (1,457)                         | \$ 10,594   | \$ (33,685)                      | \$ 114,833  |
| <b>Other Comprehensive Income (Loss)</b>  |                                    |             |                                  |             |
| Unrealized gain (loss) on available-for-sale securities,<br>net of reclassifications and income tax expense | 530                                | 132         | 1,151                            | (56)        |
| Total other comprehensive income (loss)   | 530                                | 132         | 1,151                            | (56)        |
| Comprehensive income (loss)   | \$ (927)                           | \$ 10,726   | \$ (32,534)                      | \$ 114,777  |
| Net income (loss) applicable to common stockholders   | \$ (1,457)                         | \$ 10,594   | \$ (33,685)                      | \$ 114,833  |
| Net income (loss) per share:  |                                    |             |                                  |             |
| Basic   | \$ (0.04)                          | \$ 0.33     | \$ (0.92)                        | \$ 3.60     |
| Diluted   | \$ (0.04)                          | \$ 0.30     | \$ (0.92)                        | \$ 3.29     |
| Weighted-average common shares outstanding:   |                                    |             |                                  |             |
| Basic   | 36,669                             | 32,082      | 36,518                           | 31,858      |
| Diluted   | 36,669                             | 35,272      | 36,518                           | 34,884      |

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

**REGENXBIO INC.**  
**CONSOLIDATED STATEMENTS OF STOCKHOLDERS' EQUITY**  
**(unaudited)**  
**(in thousands)**

|  | Three Months Ended June 30, 2019 |             |                                  |  |                        |                                  |
|--|----------------------------------|-------------|----------------------------------|--|------------------------|----------------------------------|
|  | Common Stock                     |             | Additional<br>Paid-in<br>Capital | Accumulated<br>Other<br>Comprehensive<br>Income (Loss) | Accumulated<br>Deficit | Total<br>Stockholders'<br>Equity |
|  | Shares                           | Amount      |                                  |  |                        |                                  |
| <b>Balances at March 31, 2019</b>  | 36,611                           | \$ 4        | \$ 602,425                       | \$ (59)  | \$ (115,317)           | \$ 487,053                       |
| Exercise of stock options  | 141                              | —           | 1,367                            | —  | —                      | 1,367                            |
| Stock-based compensation expense   | —                                | —           | 7,099                            | —  | —                      | 7,099                            |
| Unrealized gain on available-for-sale securities,<br>net of reclassifications and income tax expense | —                                | —           | —                                | 530  | —                      | 530                              |
| Net loss   | —                                | —           | —                                | —  | (1,457)                | (1,457)                          |
| <b>Balances at June 30, 2019</b>   | <u>36,752</u>                    | <u>\$ 4</u> | <u>\$ 610,891</u>                | <u>\$ 471</u>  | <u>\$ (116,774)</u>    | <u>\$ 494,592</u>                |

  

|  | Three Months Ended June 30, 2018 |             |                                  |   |                        |                                  |
|--|----------------------------------|-------------|----------------------------------|---|------------------------|----------------------------------|
|  | Common Stock                     |             | Additional<br>Paid-in<br>Capital | Accumulated<br>Other<br>Comprehensive<br>Loss | Accumulated<br>Deficit | Total<br>Stockholders'<br>Equity |
|  | Shares                           | Amount      |                                  |   |                        |                                  |
| <b>Balances at March 31, 2018</b>  | 31,900                           | \$ 3        | \$ 378,954                       | \$ (903)                                      | \$ (78,714)            | \$ 299,340                       |
| Exercise of stock options  | 375                              | —           | 3,174                            | —   | —                      | 3,174                            |
| Stock-based compensation expense   | —                                | —           | 3,982                            | —   | —                      | 3,982                            |
| Unrealized gain on available-for-sale securities,<br>net of reclassifications and income tax expense | —                                | —           | —                                | 132   | —                      | 132                              |
| Net income   | —                                | —           | —                                | —   | 10,594                 | 10,594                           |
| <b>Balances at June 30, 2018</b>   | <u>32,275</u>                    | <u>\$ 3</u> | <u>\$ 386,110</u>                | <u>\$ (771)</u>                               | <u>\$ (68,120)</u>     | <u>\$ 317,222</u>                |

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

**REGENXBIO INC.**  
**CONSOLIDATED STATEMENTS OF STOCKHOLDERS' EQUITY**  
**(unaudited)**  
**(in thousands)**

|  | Six Months Ended June 30, 2019 |             |                                  |  |                        |                                  |
|--|--------------------------------|-------------|----------------------------------|--|------------------------|----------------------------------|
|  | Common Stock                   |             | Additional<br>Paid-in<br>Capital | Accumulated<br>Other<br>Comprehensive<br>Income (Loss) | Accumulated<br>Deficit | Total<br>Stockholders'<br>Equity |
|  | Shares                         | Amount      |                                  |  |                        |                                  |
| <b>Balances at December 31, 2018</b>   | 36,120                         | \$ 4        | \$ 592,580                       | \$ (720)   | \$ (83,016)            | \$ 508,848                       |
| Adoption of ASU 2016-02 (Topic 842)  | —                              | —           | —                                | —  | (33)                   | (33)                             |
| Adoption of ASU 2018-02  | —                              | —           | —                                | 40   | (40)                   | —                                |
| Exercise of stock options  | 622                            | —           | 5,129                            | —  | —                      | 5,129                            |
| Issuance of common stock under employee<br>stock purchase plan                                       | 10                             | —           | 365                              | —  | —                      | 365                              |
| Stock-based compensation expense   | —                              | —           | 12,817                           | —  | —                      | 12,817                           |
| Unrealized gain on available-for-sale securities,<br>net of reclassifications and income tax expense | —                              | —           | —                                | 1,151  | —                      | 1,151                            |
| Net loss   | —                              | —           | —                                | —  | (33,685)               | (33,685)                         |
| <b>Balances at June 30, 2019</b>   | <u>36,752</u>                  | <u>\$ 4</u> | <u>\$ 610,891</u>                | <u>\$ 471</u>  | <u>\$ (116,774)</u>    | <u>\$ 494,592</u>                |

  

|  | Six Months Ended June 30, 2018 |             |                                  |   |                        |                                  |
|--|--------------------------------|-------------|----------------------------------|---|------------------------|----------------------------------|
|  | Common Stock                   |             | Additional<br>Paid-in<br>Capital | Accumulated<br>Other<br>Comprehensive<br>Loss | Accumulated<br>Deficit | Total<br>Stockholders'<br>Equity |
|  | Shares                         | Amount      |                                  |   |                        |                                  |
| <b>Balances at December 31, 2017</b>   | 31,295                         | \$ 3        | \$ 371,497                       | \$ (715)                                      | \$ (187,756)           | \$ 183,029                       |
| Adoption of ASU 2014-09 (Topic 606)  | —                              | —           | —                                | —   | 4,803                  | 4,803                            |
| Exercise of stock options  | 961                            | —           | 6,999                            | —   | —                      | 6,999                            |
| Issuance of common stock under employee<br>stock purchase plan                                       | 20                             | —           | 342                              | —   | —                      | 342                              |
| Stock-based compensation expense   | —                              | —           | 7,272                            | —   | —                      | 7,272                            |
| Unrealized loss on available-for-sale securities,<br>net of reclassifications and income tax expense | —                              | —           | —                                | (56)  | —                      | (56)                             |
| Net income   | —                              | —           | —                                | —   | 114,833                | 114,833                          |
| <b>Balances at June 30, 2018</b>   | <u>32,275</u>                  | <u>\$ 3</u> | <u>\$ 386,110</u>                | <u>\$ (771)</u>                               | <u>\$ (68,120)</u>     | <u>\$ 317,222</u>                |

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

**REGENXBIO INC.**  
**CONSOLIDATED STATEMENTS OF CASH FLOWS**  
**(unaudited)**  
**(in thousands)**

|   | <b>Six Months Ended June 30,</b> |                   |
|---|----------------------------------|-------------------|
|   | <b>2019</b>                      | <b>2018</b>       |
| <b>Cash flows from operating activities</b>   |                                  |                   |
| Net income (loss)   | \$ (33,685)                      | \$ 114,833        |
| Adjustments to reconcile net income (loss) to net cash provided by (used in) operating activities |                                  |                   |
| Stock-based compensation expense  | 12,817                           | 7,272             |
| Net amortization of premiums and accretion of discounts on marketable debt securities             | (788)                            | 718               |
| Depreciation and amortization   | 3,363                            | 1,730             |
| Imputed interest income from licensing  | (1,375)                          | (8,253)           |
| Unrealized gains on marketable equity securities  | (31,656)                         | —                 |
| Other non-cash adjustments  | 442                              | 13                |
| Changes in operating assets and liabilities   |                                  |                   |
| Accounts receivable   | (1,419)                          | 8,879             |
| Prepaid expenses  | (534)                            | 1,644             |
| Other current assets  | 1,419                            | (585)             |
| Operating lease right-of-use assets   | 1,156                            | —                 |
| Other assets  | (1,350)                          | (652)             |
| Accounts payable  | 1,774                            | (340)             |
| Accrued expenses and other current liabilities  | (957)                            | 2,566             |
| Deferred revenue  | (600)                            | 600               |
| Operating lease liabilities   | (1,171)                          | —                 |
| Deferred rent   | —                                | 19                |
| Other liabilities   | (482)                            | (99)              |
| Net cash provided by (used in) operating activities   | (53,046)                         | 128,345           |
| <b>Cash flows from investing activities</b>   |                                  |                   |
| Purchases of marketable securities  | (106,119)                        | (139,081)         |
| Maturities of marketable securities   | 141,262                          | 68,645            |
| Purchases of property and equipment   | (8,010)                          | (5,017)           |
| Net cash provided by (used in) investing activities   | 27,133                           | (75,453)          |
| <b>Cash flows from financing activities</b>   |                                  |                   |
| Proceeds from exercise of stock options   | 5,129                            | 6,999             |
| Proceeds from issuance of common stock under employee stock purchase plan                         | 365                              | 342               |
| Net cash provided by financing activities   | 5,494                            | 7,341             |
| Net increase (decrease) in cash and cash equivalents and restricted cash                          | (20,419)                         | 60,233            |
| <b>Cash and cash equivalents and restricted cash</b>  |                                  |                   |
| Beginning of period   | 76,614                           | 46,881            |
| End of period   | <u>\$ 56,195</u>                 | <u>\$ 107,114</u> |

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

**REGENXBIO INC.**  
**NOTES TO CONSOLIDATED FINANCIAL STATEMENTS**  
**(unaudited)**

**1. Nature of Business**

REGENXBIO Inc. (the Company) is a leading clinical-stage biotechnology company seeking to improve lives through the curative potential of gene therapy. The Company's proprietary adeno-associated virus (AAV) gene delivery platform (NAV Technology Platform) consists of exclusive rights to over 100 novel AAV vectors, including AAV7, AAV8, AAV9 and AAVrh10. The Company's NAV® Technology Platform is being applied by the Company, as well as by third-party licensees (NAV Technology Licensees), in the development of product candidates for a variety of diseases with unmet needs. The Company was formed in 2008 in the State of Delaware and is headquartered in Rockville, Maryland.

***Liquidity and Risks***

As of June 30, 2019, the Company had generated an accumulated deficit of \$116.8 million since inception. As the Company has incurred cumulative losses since inception, transition to recurring profitability is dependent upon the successful development, approval and commercialization of its product candidates and achieving a level of revenues adequate to support the Company's cost structure. The Company may never achieve recurring profitability, and unless and until it does, the Company will continue to need to raise additional capital. As of June 30, 2019, the Company had cash, cash equivalents and marketable securities of \$449.7 million, which management believes is sufficient to fund operations for at least the next 12 months from the date these consolidated financial statements were issued.

The Company is subject to risks common to companies in the biotechnology industry, including, but not limited to, development by the Company or its competitors of technological innovations, risks of failure of clinical trials, dependence on key personnel, protection of proprietary technology, compliance with government regulations and ability to transition from clinical manufacturing to the commercial production of products.

**2. Summary of Significant Accounting Policies**

***Basis of Presentation and Principles of Consolidation***

The accompanying consolidated financial statements are unaudited and have been prepared in conformity with accounting principles generally accepted in the United States of America (GAAP). The interim unaudited consolidated financial statements have been prepared on the same basis as the annual audited consolidated financial statements as of and for the year ended December 31, 2018 included in the Company's Annual Report on Form 10-K for the year ended December 31, 2018, which was filed with the SEC on February 27, 2019. Certain information and footnote disclosures required by GAAP which are normally included in the Company's annual consolidated financial statements have been omitted pursuant to SEC rules and regulations for interim reporting. In the opinion of management, the accompanying consolidated financial statements reflect all adjustments, which are normal and recurring in nature, necessary for fair financial statement presentation.

The results of operations for the interim periods are not necessarily indicative of the results of operations to be expected for the full year, any other interim periods, or any future year or period. These interim consolidated financial statements should be read in conjunction with the audited consolidated financial statements as of and for the year ended December 31, 2018, and the notes thereto, which are included in the Company's Annual Report on Form 10-K for the year ended December 31, 2018.

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

**Use of Estimates**

The preparation of financial statements in conformity with GAAP requires management to make estimates and assumptions that affect the reported amounts in the financial statements and accompanying notes. Actual results could differ materially from those estimates. Management considers many factors in selecting appropriate financial accounting policies and controls, and in developing the estimates and assumptions that are used in the preparation of these consolidated financial statements. Management must apply significant judgment in this process. In addition, other factors may affect estimates, including: expected business and operational changes, sensitivity and volatility associated with the assumptions used in developing estimates and whether historical trends are expected to be representative of future trends. The estimation process often may yield a range of potentially reasonable estimates of the ultimate future outcomes and management must select an amount that falls within that range of reasonable estimates. This process may result in actual results differing materially from those estimated amounts used in the preparation of the consolidated financial statements. Significant estimates are used in the following areas, among others: license and royalty revenue, stock-based compensation expense, accrued research and development expenses and other accrued liabilities, income taxes and the fair value of financial instruments.

**Reclassifications**

Certain amounts reported in prior periods have been reclassified to conform to current period financial statement presentation. These reclassifications are not material and have no effect on previously reported financial position, results of operations and cash flows.

**Restricted Cash**

Restricted cash includes money market mutual funds used to collateralize irrevocable letters of credit as required by the Company's lease agreements. The following table provides a reconciliation of cash and cash equivalents and restricted cash as reported on the consolidated balance sheets to the total of these amounts as reported at the end of the period in the consolidated statements of cash flows (in thousands):

|   | June 30, 2019    | June 30, 2018     |
|---|------------------|-------------------|
| Cash and cash equivalents                           | \$ 55,142        | \$ 106,889        |
| Restricted cash                                     | 1,053            | 225               |
| Total cash and cash equivalents and restricted cash | <u>\$ 56,195</u> | <u>\$ 107,114</u> |

**Marketable Securities**

Marketable securities consist of available-for-sale debt securities and equity securities and are carried at fair value. Marketable debt securities with remaining maturity dates exceeding 12 months which are not intended to be sold prior to maturity for use in current operations are classified as non-current assets. Marketable equity securities are classified as current assets.

Unrealized gains and losses on available-for-sale debt securities, net of any related tax effects, are excluded from results of operations and are included in other comprehensive income (loss) and reported as a separate component of stockholders' equity until realized. The Company uses the aggregate portfolio approach to release the tax effects of unrealized gains and losses on available-for-sale debt securities in accumulated other comprehensive income (loss). Purchase premiums and discounts on marketable debt securities are amortized or accreted into the cost basis over the life of the related security as adjustments to the yield using the effective-interest method. Interest income is recognized when earned. Unrealized gains and losses on marketable equity securities are included in results of operations as investment income. Realized gains and losses from the sale or maturity of marketable securities are based on the specific identification method and are included in results of operations as investment income.

A decline in the fair value below cost of available-for-sale debt securities that is deemed other-than-temporary is charged to results of operations, resulting in the establishment of a new cost basis for the security. The Company regularly evaluates whether declines in the fair value of its debt securities below their cost are other-than-temporary. The evaluation includes consideration of the cause of the impairment, including the creditworthiness of the security issuers, the number of securities in an unrealized loss position, the severity and duration of the unrealized losses, whether the Company has the intent to sell the securities and whether it is more likely than not that the Company will be required to sell the securities before the recovery of their amortized cost basis. The Company has not recorded any impairment of available-for-sale debt securities which was deemed to be to be other-than-temporary.

## **Leases**

Effective January 1, 2019, the Company adopted Accounting Standards Update (ASU) 2016-02, *Leases* (Topic 842) which supersedes the lease accounting requirements in Accounting Standards Codification (ASC) 840, *Leases* (Topic 840). Please refer to Recent Accounting Pronouncements below for additional information on the adoption of Topic 842 and the impact upon adoption to the Company's consolidated financial statements.

Under Topic 842, the Company classifies its leases as either finance or operating leases based on the principle of whether or not the lease is effectively a financed purchase by the Company. Lease classification is evaluated at the inception of the lease agreement. Regardless of classification, the Company records a right-of-use asset and a lease liability for all leases with a term greater than 12 months. All of the Company's leases as of June 30, 2019 have been classified as operating leases. Operating lease expense is recognized on a straight-line basis over the term of the lease, with the exception of variable lease expenses which are recognized as incurred.

The Company identifies leases in its contracts if the contract conveys the right to control the use of identified property, plant or equipment for a period of time in exchange for consideration. The Company does not allocate lease consideration between lease and nonlease components and records a lease liability equal to the present value of the remaining fixed consideration under the lease. The interest rates implicit in the Company's leases are generally not readily determinable. Accordingly, the Company uses its estimated incremental borrowing rate at the commencement date of the lease to determine the present value discount of the lease liability. The Company estimates its incremental borrowing rate for each lease based on an evaluation of its expected credit rating and the prevailing market rates for collateralized debt in a similar economic environment with similar payment terms and maturity dates commensurate with the term of the lease. The right-of-use asset for each lease is equal to the lease liability, adjusted for unamortized initial direct costs and lease incentives and prepaid or accrued rent. Initial direct costs of entering into a lease are included in the right-of-use asset and amortized as lease expense over the term of the lease. Lease incentives, such as tenant improvements allowances, are recorded as a reduction of the right-of-use asset and amortized as a reduction of lease expense over the term of the lease. The Company excludes options to extend or terminate leases from the calculation of the lease liability unless it is reasonably certain the option will be exercised.

## **Fair Value of Financial Instruments**

The Company is required to disclose information on all assets and liabilities reported at fair value that enables an assessment of the inputs used in determining the reported fair values. ASC 820, *Fair Value Measurements and Disclosures*, establishes a hierarchy of inputs used in measuring fair value that maximizes the use of observable inputs and minimizes the use of unobservable inputs by requiring that the observable inputs be used when available. Observable inputs are inputs that market participants would use in pricing the asset or liability based on market data obtained from sources independent of the Company. Unobservable inputs are inputs that reflect the Company's assumptions about the inputs that market participants would use in pricing the asset or liability, and are developed based on the best information available in the circumstances. The fair value hierarchy applies only to the valuation inputs used in determining the reported fair value of the investments and is not a measure of the investment credit quality. The three levels of the fair value hierarchy are described below:

- Level 1—Valuations based on unadjusted quoted prices in active markets for identical assets or liabilities that the Company has the ability to access at the measurement date.
- Level 2—Valuations based on quoted prices for similar assets or liabilities in markets that are not active or for which all significant inputs are observable, either directly or indirectly.
- Level 3—Valuations that require inputs that reflect the Company's own assumptions that are both significant to the fair value measurement and unobservable.

To the extent that valuation is based on models or inputs that are less observable or unobservable in the market, the determination of fair value requires more judgment. Accordingly, the degree of judgment exercised by the Company in determining fair value is greatest for instruments categorized in Level 3. A financial instrument's level within the fair value hierarchy is based on the lowest level of any input that is significant to the fair value measurement. The fair values of the Company's Level 2 instruments are based on quoted market prices or broker or dealer quotations for similar assets. These investments are initially valued at the transaction price and subsequently valued utilizing third party pricing providers or other market observable data. Please refer to Note 4 for further information on the fair value measurement of the Company's financial instruments.

### **Revenue Recognition**

The Company recognizes revenue in accordance with ASC 606, *Revenue from Contracts with Customers* (Topic 606). Topic 606 requires entities to recognize revenue when control of the promised goods or services is transferred to customers at an amount that reflects the consideration to which the entity expects to be entitled to in exchange for those goods or services. The following five steps are performed to determine the appropriate revenue recognition for arrangements within the scope of Topic 606: (i) identify the contract(s) with a customer, (ii) identify the performance obligations in the contract, (iii) determine the transaction price, (iv) allocate the transaction price to the performance obligations in the contract and (v) recognize revenue when (or as) the entity satisfies the performance obligations.

The Company applies the five-step model to contracts that are within the scope of Topic 606 only when it is probable that the Company will collect the consideration it is entitled to in exchange for the goods or services it transfers to the customer. At contract inception, for contracts within the scope of Topic 606, the Company assesses the goods or services promised within each contract and determine those that are performance obligations and whether each promised good or service is distinct. The Company then recognizes as revenue the amount of the transaction price that is allocated to respective performance obligations when (or as) the respective performance obligations are satisfied.

The Company evaluates its contracts with customers for the presence of significant financing components. If a significant financing component is identified in a contract and provides a financing benefit to the customer, the transaction price for the contract is adjusted to account for the financing portion of the arrangement, which is recognized as interest income over the financing term using the effective interest method. In determining the appropriate interest rates for significant financing components, the Company evaluates the credit profile of the customer and prevailing market interest rates and selects an interest rate in which it believes would be charged to the customer in a separate financing arrangement over a similar financing term.

### **License and Royalty Revenue**

The Company licenses its NAV Technology Platform to other biotechnology and pharmaceutical companies. The terms of the licenses vary, and licenses may be exclusive or non-exclusive and may be sublicensable by the licensee. Licenses may grant intellectual property rights for purposes of internal and preclinical research and development only, or may include the rights, or options to obtain future rights, to commercialize drug therapies for specific diseases using the Company's NAV Technology Platform. License agreements generally have a term at least equal to the life of the underlying patents, but are terminable at the option of the licensee. Consideration payable to the Company under its license agreements may include: (i) up-front and annual fees, (ii) option fees to acquire additional licenses, (iii) milestone payments based on the achievement of certain development and sales-based milestones by licensees, (iv) sublicense fees and (v) royalties on sales of licensed products.

The Company's license agreements are accounted for as contracts with customers within the scope of Topic 606. At the inception of each license agreement, the Company determines the contract term for purposes of applying the requirements of Topic 606. Licenses are generally terminable at the option of the licensee with advance notice to the Company. For each license, the Company evaluates these termination rights to determine whether a substantive termination penalty would be incurred by the licensee upon termination. If the licensee incurs a substantive termination penalty upon termination, the contract term for revenue recognition purposes is generally equal to the stated term of the license, which is the life of the underlying licensed patents. Alternatively, if the licensee does not incur a substantive termination penalty upon termination, the contract term for revenue recognition purposes may be shorter than the stated term of the license, in which case the termination rights may be accounted for as contract renewal options. The determination of whether a substantive termination penalty is associated with the termination rights requires significant judgment. In making this determination, the Company considers, among other things, the nature of the intellectual property rights that would be returned to the Company upon termination, including the exclusivity of the licensed rights and the stage of development of the licensed products, the payment terms, including the amount and timing of non-refundable or guaranteed payments, and the business purpose of the termination rights granted to the licensee. The Company considers all of the facts and circumstances relevant to each license when making this determination.

Performance obligations under the Company's license agreements may include (i) the delivery of intellectual property licenses and (ii) options granted to licensees to acquire additional licenses to the extent the options represent material rights to the licensee. At the inception of each license agreement which contains options for the licensee to acquire additional licenses, or contract renewal options, the Company evaluates the options to determine whether they provide material rights to the licensee. In making this determination, the Company considers whether the options are priced at a discount to the standalone selling price for the underlying licenses. If an option is priced at a discount to the standalone selling price for the underlying license, the option is considered to be a material right to the licensee and is accounted for as a separate performance obligation under the current license agreement.

The Company evaluates the transaction price of its license agreements at the inception of each agreement and at each reporting date. The transaction price includes the fixed consideration payable to the Company during the contract term, as well as any variable consideration to the extent that it is probable that a significant reversal of revenue will not occur in the future. Fixed consideration under the license agreements includes up-front and annual fees payable during the contract term. Variable consideration under the license agreements includes development and sales-based milestone payments, sublicense fees and royalties on sales of licensed products. Consideration contingent upon the exercise of options by a licensee is excluded from the transaction price and not accounted for as part of the license agreement until the option is exercised.

The transaction price for each license agreement is allocated to the underlying performance obligations and recognized as revenue when the performance obligations are satisfied. Consideration allocated to performance obligations for the delivery of an intellectual property license is recognized as revenue in full upon the delivery of the license to the licensee. Consideration allocated to performance obligations for license options is recognized as revenue in full upon the earlier of the option exercise or expiration. The exercise of a license option by a licensee is accounted for as a new license for revenue recognition purposes.

Up-front and annual license fees payable to the Company over the contract term of each license are included in the transaction price, and the portion of this consideration that is allocated to the performance obligation for the delivery of the intellectual property license is recognized as revenue in full upon the delivery of the license to the licensee. If annual license fees are payable to the Company in periods beyond 12 months from the delivery of the license, a significant financing component is deemed to exist which provides a financing benefit to the licensee. If a significant financing component is identified, the Company adjusts the transaction price for the license to include only the present value of the annual license fees payable to the Company over the contract term. The discounted portion of the license fees is recognized as interest income from licensing over the financing period of the license.

Development milestone payments are payable to the Company upon the achievement of specified development milestones by licensees. At the inception of each license agreement that contains development milestone payments, the Company evaluates whether the milestones are considered probable of achievement and estimates the amount to be included in the transaction price using the most likely amount method. If it is probable that a significant revenue reversal will not occur in the future, milestone payments are included in the transaction price and recognized as revenue upon the delivery of the license. Milestone payments contingent on the achievement of development milestones that are not within the control of the Company or the licensee, such as regulatory approvals, are not considered probable of being achieved and are excluded from the transaction price until the milestone is achieved. At each reporting date, the Company re-evaluates the probability of achievement of outstanding development milestones and, if necessary, adjusts the transaction price for any milestones for which the probability of achievement has changed due to current facts and circumstances. Any such adjustments are recorded on a cumulative catch-up basis and recognized as revenue in the period of the adjustment.

Royalties on sales of licensed products, sales-based milestone payments and sublicense fees based on the receipt of certain fees by licensees from any sublicensees are excluded from the transaction price of each license and recognized as revenue in the period that the related sales or sublicenses occur, provided that the associated license has been delivered to the licensee.

Royalty revenue to date consists of royalties on net sales of Zolgensma®, which is marketed by AveXis, Inc. (AveXis), a wholly owned subsidiary of Novartis AG (Novartis). Zolgensma is a licensed product under the Company's March 2014 license agreement, as amended, with AveXis for the development and commercialization of treatments for spinal muscular atrophy (SMA). The Company recognizes royalty revenue from net sales of Zolgensma in the period in which the underlying products are sold by AveXis, which in certain cases may require the Company to estimate royalty revenue for periods of net sales which have not yet been reported to the Company. Sales-based milestone payments related to net sales of Zolgensma are recognized as royalty revenue in the period in which the milestone is achieved.

The Company receives payments from licensees based on the billing schedules established in each license agreement. Amounts recognized as revenue which have not yet been received from licensees, including unbilled royalties, are recorded as accounts receivable when the Company's rights to the consideration are conditional solely upon the passage of time. Amounts recognized as revenue which have not yet been received from licensees are recorded as contract assets when the Company's rights to the consideration are not unconditional. Contract assets are recorded as other current assets on the consolidated balance sheets. If a licensee elects to terminate a license prior to the end of the license term, the licensed intellectual property is returned to the Company and any consideration recorded as accounts receivable or contract assets which is not contractually payable by the licensee is charged off as a reduction of license revenue in the period of the termination. Amounts received by the Company prior to the delivery of underlying performance obligations are deferred and recognized as revenue upon the satisfaction of the performance obligations by the Company. Deferred revenue which is not expected to be recognized within 12 months from the reporting date is recorded as non-current on the consolidated balance sheets.

### **Cost of Revenues**

Cost of revenues consists primarily of sublicense fees, milestone payments and royalties on net sales of licensed products as specified in the Company's agreements with its licensors. Sublicense fees are based on a percentage of license fees received by the Company from NAV Technology Licensees and are recognized in the period that the underlying license revenue is recognized. Milestone payments are payable to licensors upon the achievement of specified milestones by NAV Technology Licensees and are recognized in the period the milestone is achieved or deemed probable of achievement. Royalties are based on a percentage of net sales of licensed products by NAV Technology Licensees and are recognized in the period that the underlying sales occur. Amounts which are payable to licensors in periods beyond 12 months from the reporting date are recorded as non-current liabilities on the consolidated balance sheets.

### **Net Income (Loss) Per Share**

Basic net income (loss) per share is calculated by dividing net income (loss) applicable to common stockholders by the weighted-average common shares outstanding during the period, without consideration for common stock equivalents. Diluted net income (loss) per share is calculated by adjusting the weighted-average common shares outstanding for the dilutive effect of common stock equivalents outstanding for the period, determined using the treasury-stock method. Contingently convertible shares in which conversion is based on non-market-priced contingencies are excluded from the calculations of both basic and diluted net income (loss) per share until the contingency has been fully met. For purposes of the diluted net income (loss) per share calculation, common stock equivalents are excluded from the calculation of diluted net income (loss) per share if their effect would be anti-dilutive.

### **Recent Accounting Pronouncements**

#### *Adoption of ASU 2016-02, Leases*

In February 2016, the Financial Accounting Standards Board (FASB) issued ASU 2016-02, *Leases* (Topic 842) which supersedes the lease accounting requirements in ASC 840, *Leases* (Topic 840). Effective January 1, 2019, the Company adopted Topic 842 using the modified retrospective transition method. Under this method, the Company applied Topic 842 to all leases in effect as of, or entered into after, January 1, 2019 and recorded the cumulative impact of the adoption as an adjustment to its accumulated deficit on January 1, 2019. The Company's consolidated financial statements for periods ending after January 1, 2019 are presented in accordance with the requirements of Topic 842, while comparative prior period amounts have not been adjusted and continue to be reported in accordance with Topic 840. Please refer to Leases above for a description of the Company's lease accounting policies upon the adoption on Topic 842.

The Company elected certain practical expedients allowed by Topic 842 for transition purposes, including the package of practical expedients which permitted the Company to not reassess lease identification, classification and initial direct costs under Topic 842 for leases that commenced prior to January 1, 2019. Additionally, the Company elected the practical expedient allowed for transition purposes to use hindsight in determining the terms of leases that commenced prior to January 1, 2019.

Upon the adoption of Topic 842, the Company recorded operating lease right-of-use assets of \$7.4 million and operating lease liabilities of \$8.4 million for its leases which were in effect and had commenced prior to January 1, 2019 and had original lease terms of more than 12 months. The Company also derecognized current and non-current deferred rent liabilities of \$1.4 million and prepaid expenses, other current assets and other assets of \$0.4 million upon the adoption of Topic 842. Additionally, upon the adoption of Topic 842, the Company derecognized \$5.9 million of property and equipment and \$5.9 million of financing lease obligations related to construction-in-progress at 9800 Medical Center Drive, as the Company does not control the building during the construction period under the requirements of Topic 842. The lease term for the facility at 9800 Medical Center Drive does not commence until certain construction is completed by the landlord and the building is delivered to the Company. The right-of-use assets and lease liabilities related to the facility at 9800 Medical Center Drive will not be recognized on the Company's consolidated balance sheets until the commencement date of the lease, which is expected to occur in 2020.

The cumulative impact of the adoption of Topic 842 resulted in an increase in accumulated deficit of less than \$0.1 million on January 1, 2019. The adoption of Topic 842 did not have a material impact on the Company's results of operations for the three and six months ended June 30, 2019, nor does the Company believe it will have a material impact on future results of operations based on its current leasing arrangements.

### Other Recently Adopted Accounting Pronouncements

In February 2018, the FASB issued ASU 2018-02, *Income Statement—Reporting Comprehensive Income (Topic 220): Reclassification of Certain Tax Effects from Accumulated Other Comprehensive Income*, which amends the current guidance on comprehensive income to provide an option for an entity to reclassify the stranded tax effects of the Tax Cuts and Jobs Act of 2017 (the TCJA) that was signed into law in December 2017 from accumulated other comprehensive income directly to retained earnings. The stranded tax effects result from the remeasurement of deferred tax assets and liabilities which were originally recorded in comprehensive income but whose remeasurement is reflected in the income statement. The Company adopted this standard effective January 1, 2019, and upon adoption recorded a cumulative adjustment of less than \$0.1 million to reclassify the stranded tax effects of unrealized gains and losses on available-for-sale securities from accumulated other comprehensive income (loss) to accumulated deficit. The adoption of this standard did not have a material impact on the Company's financial position or results of operations.

In April 2017, the FASB issued ASU 2017-08, *Receivables—Nonrefundable Fees and Other Costs (Subtopic 310-20)*, which amends the required amortization period for certain purchased callable debt securities held at a premium by shortening the amortization period for the premium to the earliest call date. The Company adopted this standard effective January 1, 2019. The adoption of this standard required no cumulative-effect adjustments and did not have a material impact on the Company's financial position or results of operations.

### Recent Accounting Pronouncements Not Yet Adopted

In August 2018, the FASB issued ASU 2018-15, *Intangibles—Goodwill and Other—Internal-Use Software (Subtopic 350-40): Customer's Accounting for Implementation Costs Incurred in a Cloud Computing Arrangement That Is a Service Contract*. The standard aligns the requirements for capitalizing implementation costs incurred in a hosting arrangement that is a service contract with the requirements for capitalizing implementation costs incurred to develop or obtain internal-use software (and hosting arrangements that include an internal-use software license). The standard is effective for the Company beginning January 1, 2020, with early adoption permitted upon issuance, and may be applied either retrospectively or prospectively to all implementation costs incurred after the date of adoption. The Company is evaluating the application of this standard but has not yet determined the potential effects it may have on its consolidated financial statements.

In August 2018, the FASB issued ASU 2018-13, *Fair Value Measurement (Topic 820): Disclosure Framework—Changes to the Disclosure Requirements for Fair Value Measurement*, which modifies certain disclosure requirements regarding fair value measurements. The standard is effective for the Company beginning January 1, 2020, with early adoption permitted upon issuance. The Company does not believe the application of this standard will have a material impact on its financial statement disclosures.

In June 2016, the FASB issued ASU 2016-13, *Financial Instruments—Credit Losses (Topic 326): Measurement of Credit Losses on Financial Instruments*, which amends the accounting for credit losses for most financial assets and certain other instruments. The standard requires that entities holding financial assets that are not accounted for at fair value through net income be presented at the net amount expected to be collected. An allowance for credit losses will be a valuation account that will be deducted from the amortized cost basis of the financial asset to present the net carrying value at the amount expected to be collected on the financial asset. The standard is effective for the Company beginning January 1, 2020, with early adoption permitted for annual and interim periods beginning January 1, 2019. The Company does not believe the application of this standard will have a material impact on its financial position or results of operations.

### 3. Marketable Securities

The following tables present a summary of the Company's marketable securities, which consist of available-for-sale debt securities and equity securities (in thousands):

|   | Amortized<br>Cost | Unrealized<br>Gains | Unrealized<br>Losses | Fair Value        |
|---|-------------------|---------------------|----------------------|-------------------|
| <b>June 30, 2019</b>                          |                   |                     |                      |                   |
| U.S. government and federal agency securities | \$ 102,825        | \$ 388              | \$ —                 | \$ 103,213        |
| Certificates of deposit                       | 8,748             | 67                  | —                    | 8,815             |
| Corporate bonds                               | 249,377           | 1,070               | (3)                  | 250,444           |
| Equity securities                             | 420               | 31,656              | —                    | 32,076            |
|   | <u>\$ 361,370</u> | <u>\$ 33,181</u>    | <u>\$ (3)</u>        | <u>\$ 394,548</u> |

|   | Amortized<br>Cost | Unrealized<br>Gains | Unrealized<br>Losses | Fair Value        |
|---|-------------------|---------------------|----------------------|-------------------|
| <b>December 31, 2018</b>                      |                   |                     |                      |                   |
| U.S. government and federal agency securities | \$ 103,410        | \$ 93               | \$ (37)              | \$ 103,466        |
| Certificates of deposit                       | 8,992             | —                   | —                    | 8,992             |
| Corporate bonds                               | 282,902           | 36                  | (377)                | 282,561           |
|   | <u>\$ 395,304</u> | <u>\$ 129</u>       | <u>\$ (414)</u>      | <u>\$ 395,019</u> |

As of June 30, 2019 and December 31, 2018, no available-for-sale debt securities had remaining maturities greater than three years. The amortized cost of marketable debt securities is adjusted for amortization of premiums and accretion of discounts to maturity, or to the earliest call date for callable debt securities purchased at a premium.

As of June 30, 2019 and December 31, 2018, the balance in the Company's accumulated other comprehensive income (loss) consisted solely of net unrealized gains and losses on available-for-sale debt securities, net of income tax effects and reclassification adjustments for realized gains and losses. During the three and six months ended June 30, 2019, the Company recognized net unrealized gains on available-for-sale debt securities of \$0.8 million and \$1.8 million, respectively, and income tax expense of \$0.3 million and \$0.7 million, respectively, in other comprehensive income for the periods. The Company recognized net realized gains of zero and less than \$0.1 million, respectively, on the sale or maturity of available-for-sale debt securities during the three and six months ended June 30, 2019, respectively, which were reclassified out of accumulated other comprehensive income (loss) during the periods and are included in investment income in the consolidated statements of operations and comprehensive income (loss). During the three and six months ended June 30, 2018, the Company recognized net unrealized gains (losses) on available-for-sale debt securities of \$0.1 million and \$(0.1) million, respectively, and income tax expense of zero in other comprehensive income (loss) for the periods. The Company did not recognize any realized gains or losses on the sale or maturity of available-for-sale debt securities during the three and six months ended June 30, 2018. Realized gains and losses from the sale or maturity of available-for-sale debt securities are determined based on the specific identification method.

The following tables present the fair values and unrealized losses of available-for-sale debt securities held by the Company in an unrealized loss position for less than 12 months and 12 months or greater (in thousands):

|   | Less than 12 Months |                      | 12 Months or Greater |                      | Total             |                      |
|---|---------------------|----------------------|----------------------|----------------------|-------------------|----------------------|
|   | Fair Value          | Unrealized<br>Losses | Fair Value           | Unrealized<br>Losses | Fair Value        | Unrealized<br>Losses |
| <b>June 30, 2019</b>                          |                     |                      |                      |                      |                   |                      |
| Corporate bonds                               | \$ 19,176           | \$ (3)               | \$ —                 | \$ —                 | \$ 19,176         | \$ (3)               |
|   | <u>\$ 19,176</u>    | <u>\$ (3)</u>        | <u>\$ —</u>          | <u>\$ —</u>          | <u>\$ 19,176</u>  | <u>\$ (3)</u>        |
|   | Less than 12 Months |                      | 12 Months or Greater |                      | Total             |                      |
|   | Fair Value          | Unrealized<br>Losses | Fair Value           | Unrealized<br>Losses | Fair Value        | Unrealized<br>Losses |
| <b>December 31, 2018</b>                      |                     |                      |                      |                      |                   |                      |
| U.S. government and federal agency securities | \$ 53,124           | \$ (37)              | \$ —                 | \$ —                 | \$ 53,124         | \$ (37)              |
| Corporate bonds                               | 245,283             | (354)                | 12,424               | (23)                 | 257,707           | (377)                |
|   | <u>\$ 298,407</u>   | <u>\$ (391)</u>      | <u>\$ 12,424</u>     | <u>\$ (23)</u>       | <u>\$ 310,831</u> | <u>\$ (414)</u>      |

As of June 30, 2019, available-for-sale debt securities held by the Company which were in an unrealized loss position consisted of 5 investment grade security positions. The Company has the intent and ability to hold such securities until recovery and has determined that none of the securities were other-than-temporarily impaired as of June 30, 2019 or December 31, 2018.

Marketable equity securities held by the Company as of June 30, 2019 consist solely of common stock of Prevail Therapeutics Inc. (Prevail). The Company acquired the securities as consideration for a commercial license to the NAV Technology Platform granted to Prevail in August 2017. Prevail completed its initial public offering (IPO) in June 2019. Prior to Prevail's IPO, the securities were accounted for as non-marketable equity securities without a readily determinable fair value and had a carrying value of \$0.4 million. Upon Prevail's IPO in June 2019, the securities were reclassified to marketable securities and are measured at fair value. The Company recorded an unrealized gain of \$31.7 million during the three and six months ended June 30, 2019 related to the marketable equity securities of Prevail, which is included in investment income in the consolidated statements of operations and comprehensive income (loss). Pursuant to a lock-up agreement executed in connection with Prevail's IPO, the Company is restricted from selling its common stock of Prevail prior to December 2019. As of June 30, 2019, no amounts have been realized by the Company related to its marketable securities of Prevail as there have been no sales of the securities.

#### 4. Fair Value of Financial Instruments

Financial instruments reported at fair value on a recurring basis include cash equivalents and marketable securities. The following tables present the fair value of cash equivalents and marketable securities in accordance with the hierarchy discussed in Note 2 (in thousands):

|  | Quoted<br>prices<br>in active<br>markets<br>(Level 1) | Significant<br>other<br>observable<br>inputs<br>(Level 2) | Significant<br>unobservable<br>inputs<br>(Level 3) | Total             |
|--|---|---|--|-------------------|
| <b>June 30, 2019</b>                             |   |   |  |                   |
| Cash equivalents:                                |   |   |  |                   |
| Money market mutual funds                        | \$ —  | \$ 55,126   | \$ —   | \$ 55,126         |
| Total cash equivalents                           | —   | 55,126  | —  | 55,126            |
| Marketable securities:                           |   |   |  |                   |
| U.S. government and federal agency securities    | —   | 103,213   | —  | 103,213           |
| Certificates of deposit                          | —   | 8,815   | —  | 8,815             |
| Corporate bonds                                  | —   | 250,444   | —  | 250,444           |
| Equity securities                                | 32,076  | —   | —  | 32,076            |
| Total marketable securities                      | 32,076  | 362,472   | —  | 394,548           |
| Total cash equivalents and marketable securities | <u>\$ 32,076</u>                                      | <u>\$ 417,598</u>   | <u>\$ —</u>  | <u>\$ 449,674</u> |
| <b>December 31, 2018</b>                         |   |   |  |                   |
| Cash equivalents:                                |   |   |  |                   |
| Money market mutual funds                        | \$ —  | \$ 75,542   | \$ —   | \$ 75,542         |
| Total cash equivalents                           | —   | 75,542  | —  | 75,542            |
| Marketable securities:                           |   |   |  |                   |
| U.S. government and federal agency securities    | —   | 103,466   | —  | 103,466           |
| Certificates of deposit                          | —   | 8,992   | —  | 8,992             |
| Corporate bonds                                  | —   | 282,561   | —  | 282,561           |
| Total marketable securities                      | —   | 395,019   | —  | 395,019           |
| Total cash equivalents and marketable securities | <u>\$ —</u>   | <u>\$ 470,561</u>   | <u>\$ —</u>  | <u>\$ 470,561</u> |

There were no transfers of financial instruments between levels of the fair value hierarchy during the six months ended June 30, 2019.

Management estimates that the carrying amounts of its current accounts receivable, accounts payable and accrued expenses and other current liabilities approximate fair value due to the short-term nature of those instruments. Accounts receivable which contain non-current portions are recorded at their present values using a discount rate that is based on prevailing market rates and the credit profile of the licensee on the date the amounts are initially recorded. Management does not believe there have been any significant changes in market conditions or credit quality that would cause the discount rates initially used to be significantly different from those that would be used as of June 30, 2019 to determine the present value of the receivables. Accordingly, management estimates that the carrying value of its non-current accounts receivable approximates the fair value of those instruments.

Non-marketable equity securities are measured at cost less impairment, adjusted for observable price changes for identical or similar investments of the same issuer. As of December 31, 2018, non-marketable equity securities had a carrying value of \$0.4 million and were included in other assets on the consolidated balance sheets. The Company did not identify any observable price changes or changes in circumstances that would have had an adverse effect on the fair value of the securities as of December 31, 2018. In June 2019, all of the Company's non-marketable equity securities were reclassified to marketable securities and as of June 30, 2019, the Company did not hold any non-marketable equity securities. No remeasurements or impairment losses were recorded on non-marketable equity securities during the three and six months ended June 30, 2019 and 2018.

**5. Property and Equipment, Net**

Property and equipment, net consists of the following (in thousands):

|   | June 30, 2019 | December 31, 2018 |
|---|---------------|-------------------|
| Lab equipment                             | \$ 16,533     | \$ 14,417         |
| Computer equipment and software           | 2,342         | 2,002             |
| Furniture and fixtures                    | 1,946         | 1,915             |
| Leasehold improvements                    | 17,092        | 11,751            |
| Construction-in-progress                  | —             | 5,854             |
| Total property and equipment              | 37,913        | 35,939            |
| Accumulated depreciation and amortization | (10,583)      | (7,237)           |
| Property and equipment, net               | \$ 27,330     | \$ 28,702         |

Construction-in-progress reported in the table above as of December 31, 2018 consisted of certain costs incurred and reported by the Company's landlord at 9800 Medical Center Drive. Upon the adoption of Topic 842 on January 1, 2019, the Company derecognized the cumulative amount of construction costs incurred by the landlord of \$5.9 million. Please refer to Note 2 for further information on the Company's adoption of Topic 842 and Note 6 for further information on the Company's lease at 9800 Medical Center Drive.

**6. Leases*****9800 Medical Center Drive***

In November 2018, the Company entered into a lease agreement, as amended in April 2019, for approximately 139,000 square feet of office and laboratory facilities in a new building to be constructed at 9800 Medical Center Drive in Rockville, Maryland (the 9800 Medical Center Drive Lease). The initial construction of the building is being conducted by the landlord and is expected to be completed in 2020, after which the leased premises will be delivered to the Company to make additional improvements to the building. Pursuant to the amended lease agreement, the Company will receive a \$15.3 million tenant improvement allowance from the landlord to construct additional improvements to the leased premises. The lease expires approximately 16 years from delivery of the leased premises to the Company, subject to certain extension and termination options held by the Company. The Company has the option to extend the term of the lease for up to 10 additional years and the option to terminate the lease after 12 years from the delivery of the leased premises to the Company. If the Company elects to terminate the lease, it will be subject to a termination fee equal to the unamortized tenant improvement allowance, rent abatement and landlord commissions as of the termination date, bearing interest at 5% per annum, plus four months of base rent and operating expenses. Additionally, after the delivery of the leased premises under the 9800 Medical Center Drive Lease, the Company will have the option to terminate its lease at 9712 Medical Center Drive with six months' notice. Monthly payments under the 9800 Medical Center Drive Lease begin approximately 12 months from the delivery of the leased premises to the Company and escalate annually in accordance with the lease agreement. As required by the lease agreement, the Company has provided the landlord with an irrevocable letter of credit of \$0.8 million which the landlord may draw upon in the event of any uncured default by the Company under the terms of the lease.

The Company is involved in the construction project for the leased premises at 9800 Medical Center Drive, including having the responsibility to pay for a portion of the costs of non-normal tenant improvements such as finish work, mechanical, electrical and plumbing elements of the building, among other items. As of December 31, 2018, under the requirements of Topic 840, the Company was deemed the owner of the leased premises during the construction period for accounting purposes and certain estimated construction costs incurred and reported by the landlord were recorded as property and equipment, with a corresponding financing lease obligation, on the consolidated balance sheet. The Company has determined that it does not control the building during the construction period under the requirements of Topic 842. Accordingly, upon the adoption of Topic 842 on January 1, 2019, the Company derecognized property and equipment of \$5.9 million for the cumulative costs of construction incurred by the landlord as well as the associated \$5.9 million financing lease obligation. As of June 30, 2019, the Company had recorded \$4.8 million of costs related to construction-in-progress at 9800 Medical Center Drive, which have been recorded as leasehold improvements within property and equipment on the consolidated balance sheets.

As of June 30, 2019, the right-of-use assets and lease liabilities related to the 9800 Medical Center Drive Lease have not been recorded on the Company's consolidated balance sheets and will be measured and recognized on the commencement date of the lease, which is expected to occur in 2020 when the landlord delivers the newly constructed building to the Company.

### **Other Leases**

In March 2015, the Company entered into an operating lease for office space at 9712 Medical Center Drive in Rockville, Maryland (the 9712 Medical Center Drive Lease). The lease term commenced in April 2015. Monthly payments under the lease began in October 2015 and escalate annually in accordance with the lease agreement.

In September 2015, November 2015, July 2017 and April 2018, the Company amended the 9712 Medical Center Drive Lease to include additional office and laboratory space at 9714 Medical Center Drive, and ultimately extend the term of the lease to September 2021. The Company has options to extend the term of the 9712 Medical Center Drive Lease for up to six additional years. Additionally, upon the commencement of the 9800 Medical Center Drive Lease, the Company will have the option to terminate the 9712 Medical Center Drive Lease with six months' notice. The Company's extension and termination options under the 9712 Medical Center Drive Lease have been excluded from the measurement of the right-of-use assets and lease liabilities for the lease as they are not reasonably certain of exercise. The Company received a \$0.4 million tenant improvement allowance from the landlord which has been recorded as a reduction of the right-of-use assets for the lease and is amortized on a straight-line basis as a reduction of rent expense over the term of the lease.

In January 2016, the Company entered into an operating lease for its corporate headquarters at 9600 Blackwell Road in Rockville, Maryland (the Blackwell Road Lease). The lease commenced in February 2016 and expires in September 2023. In November 2017, the Blackwell Road Lease was amended to include additional office space for the remainder of the lease term. Monthly payments under the lease began in September 2016 and escalate annually in accordance with the lease agreement. The Company has an option to extend the term of the Blackwell Road Lease for up to five additional years and the option to terminate the lease after 67 months from the lease commencement date. If the Company elects to terminate the lease, it will be subject to a termination fee equal to the unamortized tenant improvement allowance, rent abatement and landlord costs and commissions as of the termination date, bearing interest at 8% per annum. The Company's extension and termination options under the Blackwell Road Lease have been excluded from the measurement of the right-of-use assets and lease liabilities for the lease as they are not reasonably certain of exercise. The Company received a \$0.8 million tenant improvement allowance from the landlord which has been recorded as a reduction of the right-of-use assets for the lease and is amortized on a straight-line basis as a reduction of rent expense over the term of the lease.

In May 2016, the Company entered into an operating lease for office space at 400 Madison Avenue in New York, New York (the 400 Madison Lease). The lease commenced in July 2016 and monthly payments under the lease began in October 2016 and escalate annually in accordance with the lease agreement. As required by the lease agreement, the Company has provided the landlord with an irrevocable letter of credit of \$0.2 million which the landlord may draw upon in the event of any uncured default by the Company under the terms of the lease.

In May 2019, the 400 Madison Lease was amended to include additional office space and extend the term of the lease. Prior to the amendment, the lease was to expire in October 2020. Pursuant to the amendment, the Company will vacate the original leased premises in the third quarter of 2019, upon which the landlord will perform demolition of the original premises as well as the additional premises under the amended lease. Upon completion of the demolition, the landlord will deliver the entire expanded premises to the Company to make tenant improvements. The amended lease will expire approximately 7.5 years from the date the expanded premises are delivered to the Company, which is expected to occur in 2019. The Company will receive a \$0.7 million tenant improvement allowance from the landlord to construct improvements to the leased premises.

As a result of the amendment in May 2019, the expiration of the lease term for the original premises under the 400 Madison Lease was adjusted from October 2020 to August 2019. Accordingly, the right-of-use assets and lease liabilities for the original premises were reduced by \$0.4 million to account for the modification of the lease term. As of June 30, 2019, the Company does not have control of the additional leased premises under the amended lease and the right-of-use assets and lease liabilities have not been recorded on the Company's consolidated balance sheets. The right-of-use assets and lease liabilities related to the expanded premises for the extended lease term will be measured and recognized upon the delivery of the expanded premises to the Company to make tenant improvements.

The Company leases additional office and laboratory facilities in Rockville, Maryland, as well as laboratory and other equipment, under operating leases with various expiration dates through 2022.

### Operating Lease Information

All of the Company's leases are classified as operating leases. The following table summarizes the Company's lease costs and supplemental cash flow information related to operating leases (in thousands):

|  | Three Months Ended<br>June 30, 2019 | Six Months Ended<br>June 30, 2019 |
|--|-------------------------------------|-----------------------------------|
| Operating lease cost   | \$ 697                              | \$ 1,398                          |
| Variable lease cost  | 138                                 | 298                               |
| Total lease cost   | <u>\$ 835</u>                       | <u>\$ 1,696</u>                   |
| Cash paid for amounts included in operating lease liabilities    | \$ 684                              | \$ 1,395                          |
| Right-of-use assets acquired through operating lease liabilities | \$ (371)                            | \$ (335)                          |

Right-of-use assets acquired through operating lease liabilities for the three and six months ended June 30, 2019 includes a reduction of \$0.4 million related to the April 2019 amendment to the 400 Madison Lease for the adjustment of the lease term. Short-term lease expense for the three and six months ended June 30, 2019 was not material and is included in operating lease cost in the table above. Variable lease cost under the Company's operating leases includes items such as common area maintenance, utilities, taxes and other charges.

The weighted-average remaining lease term and weighted-average discount rate of the Company's operating leases were as follows:

|   | As of<br>June 30, 2019 |
|---|------------------------|
| Weighted-average remaining lease term (years) | 2.9                    |
| Weighted-average discount rate                | 5.6%                   |

The following table presents a reconciliation of the undiscounted future minimum lease payments remaining under leases that have not yet commenced and other operating leases to the amounts reported as operating lease liabilities on the consolidated balance sheet as of June 30, 2019 (in thousands):

|  | Leases<br>Not Yet<br>Commenced (a) | Other<br>Operating<br>Leases | Total<br>Minimum Lease<br>Payments |
|--|------------------------------------|------------------------------|------------------------------------|
| Undiscounted future minimum lease payments:      |                                    |                              |                                    |
| 2019 (remainder of year)                         | \$ 98                              | \$ 1,200                     | \$ 1,298                           |
| 2020   | 460                                | 2,813                        | 3,273                              |
| 2021   | 2,308                              | 2,412                        | 4,720                              |
| 2022   | 5,422                              | 623                          | 6,045                              |
| 2023   | 6,376                              | 479                          | 6,855                              |
| Thereafter                                       | 83,650                             | —                            | 83,650                             |
| Total undiscounted future minimum lease payments | <u>\$ 98,314</u>                   | <u>\$ 7,527</u>              | <u>\$ 105,841</u>                  |
| Amount representing imputed interest             |                                    | (597)                        |                                    |
| Total operating lease liabilities                |                                    | 6,930                        |                                    |
| Current portion of operating lease liabilities   |                                    | (2,276)                      |                                    |
| Operating lease liabilities, non-current         |                                    | <u>\$ 4,654</u>              |                                    |

(a) Includes undiscounted future minimum lease payments under the 9800 Medical Center Drive Lease and 400 Madison Lease which are not included in the lease liabilities reported on the consolidated balance sheet as of June 30, 2019. The actual timing and amounts of these payments are subject to adjustment based on the commencement dates and actual square footage of the leased premises. Accordingly, these amounts were estimates as of June 30, 2019.

As of December 31, 2018, future minimum lease payments under Topic 840 for the 9800 Medical Center Drive Lease and other operating leases were as follows (in thousands):

|                                     | 9800 Medical<br>Center Drive<br>Lease (a) | Other<br>Operating<br>Leases | Total<br>Minimum Lease<br>Payments |
|-------------------------------------|---|------------------------------|------------------------------------|
| 2019                                | \$ —                                      | \$ 2,798                     | \$ 2,798                           |
| 2020                                | —   | 3,054                        | 3,054                              |
| 2021                                | 1,329                                     | 2,391                        | 3,720                              |
| 2022                                | 4,289                                     | 621                          | 4,910                              |
| 2023                                | 5,156                                     | 479                          | 5,635                              |
| Thereafter                          | 76,420                                    | —                            | 76,420                             |
| <b>Total minimum lease payments</b> | <b>\$ 87,194</b>                          | <b>\$ 9,343</b>              | <b>\$ 96,537</b>                   |

- (a) Includes all future minimum lease payments under the 9800 Medical Center Drive Lease, including amounts recorded as financing lease obligations on the consolidated balance sheet. The actual timing and amounts of payments under the 9800 Medical Center Drive Lease are subject to adjustment based on the commencement date and actual square footage of the leased premises. Accordingly, these amounts were estimates as of December 31, 2018.

## 7. Commitments and Contingencies

### *License from The Trustees of the University of Pennsylvania*

In February 2009, the Company entered into a license agreement, which has been amended from time to time, with The Trustees of the University of Pennsylvania (together with the University of Pennsylvania, Penn) for exclusive, worldwide rights to certain patents owned by Penn underlying the Company's NAV Technology Platform, as well as exclusive rights to certain data, results and other information generated in connection with the clinical trial for RGX-501, the Company's product candidate for the treatment of homozygous familial hypercholesterolemia (HoFH). Pursuant to the license agreement, the Company is obligated to pay Penn royalties on net sales of licensed products and sublicense fees. Additionally, the Company is obligated to reimburse Penn for certain costs incurred related to the maintenance of the licensed patents.

In April 2019, the Company amended its license from Penn to include exclusive license rights to certain know-how, including research data and other information, relating to the treatment of late-infantile neuronal ceroid lipofuscinosis type 2 (CLN2) disease. In consideration for the additional licensed rights, and in addition to any consideration owed under the license prior to the amendment, the Company paid Penn an upfront fee and is obligated to pay milestone fees of up to \$20.5 million upon the achievement of various development and sales-based milestones and additional royalties on net sales of licensed products for the treatment of CLN2 disease. Additionally, the amendment modifies the percentage of sublicense fees the Company is obligated to pay Penn on amounts the Company receives from third parties for the sublicensing of the licensed rights for the treatment of CLN2 disease.

### *European Patent Office Proceeding*

In June 2017, a third party filed an opposition with the European Patent Office (EPO) challenging the validity of a European patent owned by Penn for the AAV8 vector, which the Company has exclusively licensed (EU AAV8 Patent). The EPO conducted oral proceedings in October 2018 and upheld the validity of the EU AAV8 Patent subject to certain amendments made during the proceeding. Each party to the proceeding has appealed the EPO's ruling. As of June 30, 2019 and December 31, 2018, the Company had not recorded any liabilities related to this matter nor does the Company believe this matter will have a material adverse impact on its business.

## 8. License and Royalty Revenue

As of June 30, 2019, the Company's NAV Technology Platform was being applied in the development of more than 20 partnered product candidates by nine NAV Technology Licensees. Consideration to the Company under its license agreements may include: (i) up-front and annual fees, (ii) option fees to acquire additional licenses, (iii) milestone payments based on the achievement of certain development and sales-based milestones by licensees, (iv) sublicense fees and (v) royalties on sales of licensed products. Sublicense fees vary by license and range from a mid-single digit percentage to a low-double digit percentage of license fees received by licensees as a result of sublicenses. Royalties on net sales of commercialized products vary by license and range from a mid-single digit percentage to a low double-digit percentage of net sales by licensees.

Development milestone payments are only included in the transaction price of each license and recognized as revenue to the extent they are considered probable of achievement. Sales-based milestones are excluded from the transaction price of each license agreement and recognized as revenue in the period of achievement. As of June 30, 2019, the Company's license agreements, excluding additional licenses that could be granted upon the exercise of options by licensees, contained unachieved milestones which could result in aggregate milestone payments to the Company of up to \$18.6 million upon the commencement of various stages of clinical trials, \$32.5 million upon the submission of regulatory approval filings, \$81.0 million upon the approval of commercial products by regulatory agencies and \$187.0 million upon the achievement of specified sales targets for licensed products. To the extent the milestone payments are realized by the Company, the Company will be obligated to pay sublicense fees to licensors based on a specified percentage of the fees earned by the Company. The achievement of milestones by licensees is highly dependent on the successful development and commercialization of licensed products and it is at least reasonably possible that some or all of the milestone fees will not be realized by the Company.

The following tables present changes in the balances of the Company's receivables, contract assets and contract liabilities during the periods presented (in thousands):

|  | Balance at<br>Beginning<br>of Period | Net Additions<br>(Deductions) | Balance at<br>End of Period |
|--|--------------------------------------|-------------------------------|-----------------------------|
| <b>Three Months Ended June 30, 2019</b>      |                                      |                               |                             |
| Receivables and contract assets:             |                                      |                               |                             |
| Accounts receivable, current and non-current | \$ 31,130                            | \$ 2,504                      | \$ 33,634                   |
| Contract assets                              | \$ 1,000                             | \$ (1,000)                    | \$ —                        |
| Contract liabilities:                        |                                      |                               |                             |
| Deferred revenue, current and non-current    | \$ 3,933                             | \$ (600)                      | \$ 3,333                    |

|  | Balance at<br>Beginning<br>of Period | Net Additions<br>(Deductions) | Balance at<br>End of Period |
|--|--------------------------------------|-------------------------------|-----------------------------|
| <b>Six Months Ended June 30, 2019</b>        |                                      |                               |                             |
| Receivables and contract assets:             |                                      |                               |                             |
| Accounts receivable, current and non-current | \$ 31,599                            | \$ 2,035                      | \$ 33,634                   |
| Contract assets                              | \$ 750                               | \$ (750)                      | \$ —                        |
| Contract liabilities:                        |                                      |                               |                             |
| Deferred revenue, current and non-current    | \$ 3,933                             | \$ (600)                      | \$ 3,333                    |

|  | Balance at<br>Beginning<br>of Period | Net Additions<br>(Deductions) | Balance at<br>End of Period |
|--|--------------------------------------|-------------------------------|-----------------------------|
| <b>Three Months Ended June 30, 2018</b>      |                                      |                               |                             |
| Receivables and contract assets:             |                                      |                               |                             |
| Accounts receivable, current and non-current | \$ 58,621                            | \$ (53,397)                   | \$ 5,224                    |
| Contract assets                              | \$ 250                               | \$ (250)                      | \$ —                        |
| Contract liabilities:                        |                                      |                               |                             |
| Deferred revenue, current and non-current    | \$ —                                 | \$ 600                        | \$ 600                      |

|  | Balance at<br>Beginning<br>of Period | Net Additions<br>(Deductions) | Balance at<br>End of Period |
|--|--------------------------------------|-------------------------------|-----------------------------|
| <b>Six Months Ended June 30, 2018</b>        |                                      |                               |                             |
| Receivables and contract assets:             |                                      |                               |                             |
| Accounts receivable, current and non-current | \$ 5,850                             | \$ (626)                      | \$ 5,224                    |
| Contract assets                              | \$ 350                               | \$ (350)                      | \$ —                        |
| Contract liabilities:                        |                                      |                               |                             |
| Deferred revenue, current and non-current    | \$ —                                 | \$ 600                        | \$ 600                      |

The net increases in accounts receivable during the three and six months ended June 30, 2019 were primarily attributable to additional licenses granted by the Company during the periods as a result of license options exercised by licensees. The net decrease in accounts receivable during the three months ended June 30, 2018 was primarily attributable to the acceleration and collection of \$100.0 million in license fees under our amended March 2014 license agreement with AveXis as a result of their acquisition by Novartis in May 2018. Of the \$100.0 million received, \$53.3 million was previously recorded as accounts receivable at the beginning of the period.

As of June 30, 2019, the Company had recorded deferred revenue, current and non-current, of \$3.3 million which represents consideration received from licensees for performance obligations that have not yet been satisfied by the Company. Unsatisfied performance obligations consist of options granted to licensees that provide material rights to the licensee to acquire additional licenses from the Company. These performance obligations will be satisfied, and underlying revenue will be recognized, upon the exercise or expiration of the options. During the three and six months ended June 30, 2019, the Company recognized \$0.6 million of license revenue that was previously included in deferred revenue at the beginning of the period as a result of options exercised by licensees during the period. The Company did not recognize any license revenue during the three and six months ended June 30, 2018 that was included in deferred revenue at the beginning of the period.

During the three and six months ended June 30, 2019, the Company recognized license revenue of \$3.2 million and \$4.0 million, respectively, from licenses delivered to licensees in prior periods as a result of changes in the transaction prices of its license agreements. Changes in the transaction prices during the three and six months ended June 30, 2019 were primarily attributable to development milestones achieved or deemed probable of achievement during the period that were previously not considered probable of achievement. During the three and six months ended June 30, 2018, the Company recognized license revenue of \$39.9 million and \$0.2 million, respectively, from licenses delivered to licensees in prior periods as a result of changes in the transaction prices of its license agreements. Changes in the transaction prices during the three and six months ended June 30 2018 were primarily attributable to development milestones achieved or deemed probable of achievement during the period that were previously not considered probable of achievement, as well as the acquisition of AveXis by Novartis in May 2018 resulting in the accelerated payment of \$40.0 million of sales-based milestones which were previously excluded from the transaction price of the license.

As of June 30, 2019, the Company had recorded total current and non-current accounts receivable of \$33.6 million, of which \$0.2 million had been billed to customers and \$33.4 million was billable to customers in future periods. As of December 31, 2018, the Company had recorded total current and non-current accounts receivable of \$31.6 million, of which \$0.4 million had been billed to customers and \$31.2 million was billable to customers in future periods.

Accounts receivable, current and non-current, as of June 30, 2019 and December 31, 2018 included \$27.3 million and \$26.0 million, respectively, related to the November 2018 license agreement with Abeona Therapeutics Inc. for the development and commercialization of treatments for various diseases. The Company believes that it is not exposed to significant credit risk related to accounts receivable due to the credit quality and history of collections from its significant customers. The Company is unaware of any concentrations of credit risk related to accounts receivable from significant customers with deteriorated credit quality. As of June 30, 2019 and December 31, 2018, the Company had not recognized any impairment losses on its receivables or contract assets from contracts with customers and no allowance for doubtful accounts was recorded.

#### ***AveXis March 2014 License and January 2018 Amendment***

In March 2014, the Company entered into an exclusive license agreement (the March 2014 License) with AveXis. Under the license, the Company granted AveXis an exclusive, worldwide commercial license, with rights to sublicense, to the NAV AAV9 vector for the treatment of SMA in humans by *in vivo* gene therapy. In consideration for the license, AveXis paid the Company an up-front fee of \$2.0 million, and is required to pay annual fees, development milestone payments of up to \$12.3 million, mid-single to low double-digit royalties on net sales of licensed products, subject to reduction in specified circumstances, and a lower mid-double digit percentage of any sublicense fees AveXis receives from sublicensees for the licensed intellectual property rights.

In January 2018, the Company and AveXis amended the March 2014 License (the January 2018 Amendment). Under the January 2018 Amendment, the licensed intellectual property was expanded to include, in addition to the NAV AAV9 vector previously licensed, sublicenses to other third-party patents exclusively licensed by the Company as well as any other recombinant AAV vector in the Company's intellectual property portfolio during a period of 14 years from the effective date of the January 2018 Amendment, for the treatment of SMA in humans by *in vivo* gene therapy. The Company may also, in its sole discretion, provide specified collaborative services to AveXis as specified in the January 2018 Amendment.

The January 2018 Amendment also modified the assignment provision of the March 2014 License. Under the amended assignment provision, AveXis was permitted to transfer the March 2014 License, as amended, without the Company's consent in connection with a change of control of AveXis, subject to certain conditions. Under the original March 2014 License, any assignment by AveXis without the Company's prior written consent had been prohibited.

In consideration for the additional rights granted under the January 2018 Amendment, and in addition to any consideration owed under the original March 2014 License, AveXis paid to the Company a fee of \$80.0 million upon entry into the January 2018 Amendment. In addition, AveXis was obligated to pay the Company (i) \$30.0 million on the first anniversary of the effective date of the January 2018 Amendment, (ii) \$30.0 million on the second anniversary of the effective date of the January 2018 Amendment and (iii) potential sales-based milestone payments of up to \$120.0 million. In the event of a change of control of AveXis, to the extent that any fee described in (i) or (ii) above, or the first \$40.0 million of sales-based milestone payments described in (iii) above, had not yet been paid to the Company, AveXis was required to pay any such unpaid fee to the Company upon the change of control. For any product developed for the treatment of SMA using the NAV AAV9 vector, AveXis will continue to be obligated to pay to the Company mid-single to low double-digit royalties on net sales as required by the March 2014 License, and for any product developed for the treatment of SMA using a licensed vector other than NAV AAV9, the Company will receive a low double-digit royalty on net sales.

In May 2018, AveXis was acquired by Novartis, which qualified as a change of control of AveXis under the January 2018 Amendment. Pursuant to the January 2018 Amendment, AveXis paid the Company \$100.0 million in accelerated license payments as a result of the change of control.

In May 2019, the U.S. Food and Drug Administration (the FDA) approved Zolgensma for marketing in the United States, which is a licensed product under the March 2014 License, as amended, with AveXis. Upon its commercial launch in the second quarter of 2019, the Company began recognizing royalty revenue on net sales of Zolgensma.

#### *Accounting Analysis*

The January 2018 Amendment was accounted for under Topic 606 as a modification of the license agreement resulting in a new and separate contract from the original March 2014 License for revenue recognition purposes. The Company determined that a substantive termination penalty is associated with AveXis' termination rights under the amended license agreement, and therefore the contract term for revenue recognition purposes is equal to the stated term of the license. The only material performance obligation of the Company under the January 2018 Amendment is for the delivery of the modified license, which occurred upon the execution of the amendment in January 2018.

As of June 30, 2019, the transaction price of the original March 2014 License was \$11.0 million. The transaction price includes (i) the up-front payment in March 2014 of \$2.0 million, (ii) the present value of aggregate annual fees payable to the Company over the term of the license and (iii) payments for development milestones achieved to date or which are deemed probable of achievement. The discounted portion of the annual fees represents the financing benefit provided to AveXis and is recognized as interest income from licensing over the term of the license. Variable consideration under the original March 2014 License, which has been excluded from the transaction price, includes \$3.5 million in payments for remaining development milestones that had not yet been achieved and were not considered probable of achievement, as well as any potential sublicense fees or royalties on sales of licensed products, which will be recognized in the period of the underlying sales or sublicenses. The transaction price of the original March 2014 License increased by \$3.5 million during the three and six months ended June 30, 2019 as a result of development milestones achieved during the period which were previously excluded from the transaction price.

Upon its execution, the transaction price of the January 2018 Amendment was \$132.1 million, which was fully recognized as license revenue upon the delivery of the modified license in January 2018. In May 2018, as a result of the acquisition of AveXis by Novartis, the transaction price was increased by \$40.0 million to account for the acceleration of the sale-based milestone which was previously excluded from the transaction price. The \$40.0 million increase in the transaction price was recognized as license revenue upon the completion of the change of control in May 2018 since the amended license had been fully delivered to AveXis. Additionally, due to the acceleration of the two \$30.0 million payments originally due in January 2019 and January 2020, the Company recognized \$6.1 million of interest income from licensing upon the completion of the change of control of AveXis, which represents the remaining present value discount on such payments as of the date of the change of control. As of June 30, 2019, the transaction price of the January 2018 Amendment was \$172.1 million, which includes: (i) the \$80.0 million payment in January 2018, (ii) the present value, as of the date of the January 2018 Amendment, of the two \$30.0 million payments originally due in January 2019 and January 2020 and (iii) the \$40.0 million sales-based milestone which was accelerated upon the change of control in May 2018. Variable consideration under the January 2018 Amendment, which has been excluded from the transaction price, includes the remaining sales-based milestone payment of \$80.0 million, as well as any potential sublicense fees or royalties on sales of licensed products, which will be recognized in the period of the underlying sales or sublicenses. There were no increases in the transaction price of the January 2018 Amendment during the three and six months ended June 30, 2019.

The Company recognized the following amounts under the March 2014 License with AveXis, as amended, which include amounts from both the original March 2014 License and the January 2018 Amendment (in thousands):

|                                | Three Months Ended June 30, |           | Six Months Ended June 30, |            |
|--------------------------------|-----------------------------|-----------|---------------------------|------------|
|                                | 2019                        | 2018      | 2019                      | 2018       |
| License and royalty revenue    | \$ 4,424                    | \$ 40,000 | \$ 4,424                  | \$ 172,066 |
| Interest income from licensing | \$ 7                        | \$ 6,752  | \$ 15                     | \$ 7,950   |

As of June 30, 2019, the Company had recorded \$1.1 million of accounts receivable from AveXis under the March 2014 License, as amended, of which \$0.9 million were included in current assets and \$0.2 million were included in non-current assets. As of December 31, 2018, the Company had recorded \$0.2 million of accounts receivable from AveXis under the March 2014 License, as amended, of which less than \$0.1 million were included in current assets and \$0.2 million were included in non-current assets.

## 9. Stock-based Compensation

In January 2019, an additional 1,444,808 shares became available for issuance under the 2015 Equity Incentive Plan (the 2015 Plan). As of June 30, 2019, the total number of shares of common stock authorized for issuance under the 2015 Plan and the 2014 Stock Plan (the 2014 Plan) was 10,933,221, of which 2,327,843 remained available for future grants under the 2015 Plan.

### Stock-based Compensation Expense

The Company's stock-based compensation expense by award type was as follows (in thousands):

|                              | Three Months Ended June 30, |                 | Six Months Ended June 30, |                 |
|------------------------------|-----------------------------|-----------------|---------------------------|-----------------|
|                              | 2019                        | 2018            | 2019                      | 2018            |
| Stock options                | \$ 6,844                    | \$ 3,818        | \$ 12,296                 | \$ 6,939        |
| Restricted stock units       | 69                          | 69              | 136                       | 136             |
| Employee stock purchase plan | 186                         | 95              | 385                       | 197             |
|                              | <u>\$ 7,099</u>             | <u>\$ 3,982</u> | <u>\$ 12,817</u>          | <u>\$ 7,272</u> |

As of June 30, 2019, the Company had \$70.4 million of unrecognized stock-based compensation expense related to stock options, restricted stock units and the 2015 Employee Stock Purchase Plan (the 2015 ESPP), which is expected to be recognized over a weighted-average period of 3.0 years.

The Company has recorded aggregate stock-based compensation expense in the consolidated statements of operations and comprehensive income (loss) as follows (in thousands):

|                            | Three Months Ended June 30, |                 | Six Months Ended June 30, |                 |
|----------------------------|-----------------------------|-----------------|---------------------------|-----------------|
|                            | 2019                        | 2018            | 2019                      | 2018            |
| Research and development   | \$ 3,526                    | \$ 1,895        | \$ 5,873                  | \$ 3,424        |
| General and administrative | 3,573                       | 2,087           | 6,944                     | 3,848           |
|                            | <u>\$ 7,099</u>             | <u>\$ 3,982</u> | <u>\$ 12,817</u>          | <u>\$ 7,272</u> |

### Stock Options

The following table summarizes stock option activity under the 2014 Plan and 2015 Plan (in thousands, except per share data):

|  | Shares       | Weighted-<br>average<br>Exercise<br>Price | Weighted-<br>average<br>Remaining<br>Contractual<br>Life<br>(Years) | Aggregate<br>Intrinsic<br>Value (a) |
|--|--------------|---|---|-------------------------------------|
| Outstanding at December 31, 2018             | 4,855        | \$ 19.31                                  | 7.6   | \$ 118,185                          |
| Granted                                      | 1,410        | \$ 48.48                                  |   |                                     |
| Exercised                                    | (621)        | \$ 8.24                                   |   |                                     |
| Cancelled or forfeited                       | (190)        | \$ 32.92                                  |   |                                     |
| Outstanding at June 30, 2019                 | <u>5,454</u> | \$ 27.64                                  | 7.8   | \$ 136,336                          |
| Exercisable at June 30, 2019                 | <u>2,630</u> | \$ 13.65                                  | 6.7   | \$ 99,341                           |
| Vested and expected to vest at June 30, 2019 | <u>5,454</u> | \$ 27.64                                  | 7.8   | \$ 136,336                          |

- (a) The aggregate intrinsic value is calculated as the difference between the exercise price of the underlying options and the fair value of the common stock for the options that were in the money at the dates reported.

The weighted-average grant date fair value per share of options granted during the six months ended June 30, 2019 was \$32.24. During the six months ended June 30, 2019, the total number of stock options exercised was 621,415, resulting in total proceeds of \$5.1 million. The total intrinsic value of options exercised during the six months ended June 30, 2019 was \$25.5 million.

### Restricted Stock Units

The following table summarizes restricted stock unit activity under the 2015 Plan (in thousands, except per share data):

|                                       | Shares    | Weighted-<br>average<br>Grant Date<br>Fair Value |
|---------------------------------------|-----------|--|
| Unvested balance at December 31, 2018 | 40        | \$ 20.90   |
| Granted                               | —         | \$ —   |
| Vested                                | —         | \$ —   |
| Forfeited                             | —         | \$ —   |
| Unvested balance at June 30, 2019     | <u>40</u> | \$ 20.90   |

### Employee Stock Purchase Plan

As of June 30, 2019, the total number of shares of common stock authorized for issuance under the 2015 ESPP was 254,000, of which 159,339 remained available for future issuance. During the six months ended June 30, 2019, 10,241 shares of common stock were issued under the 2015 ESPP.

## 10. Income Taxes

The Company has evaluated the positive and negative evidence bearing upon the realizability of its deferred tax assets. Based on the Company's history of operating losses, including a three-year cumulative loss position as of June 30, 2019 and December 31, 2018, the Company has concluded that it is more likely than not that the benefit of its deferred tax assets will not be realized. Accordingly, the Company has provided a full valuation allowance for its net deferred tax assets as of June 30, 2019 and December 31, 2018.

During the three and six months ended June 30, 2019, the Company recognized income tax benefit of \$0.1 million and \$0.2 million, respectively, and income tax expense in other comprehensive income of \$0.3 million and \$0.7 million, respectively, related to net unrealized gains on available-for-sale debt securities. As of June 30, 2019, the Company had accrued \$0.5 million related to this tax benefit, which is expected to be generated from losses in continuing operations in 2019 and is included in accrued expenses and other current liabilities on the consolidated balance sheet.

## 11. Related Party Transactions

### FOKKISER LLP

Since 2016, the Company has been party to professional services agreements with FOKKISER LLP (FOKKISER), an affiliate of certain stockholders of the Company and an affiliate of a member of the Company's Board of Directors, pursuant to which the Company pays a fixed monthly fee in consideration for certain strategic services provided by FOKKISER. Effective January 2019, the Company entered into a new professional services agreement with FOKKISER with similar terms and conditions as the previous agreements. The agreement was amended effective June 2019 to expand the services provided and increase the monthly fee, and the amended agreement expires in December 2020. Expenses incurred under the agreements with FOKKISER for the three and six months ended June 30, 2019 were \$0.9 million and \$1.7 million, respectively. Expenses incurred under the agreements with FOKKISER for the three and six months ended June 30, 2018 were \$0.5 million and \$1.1 million, respectively. Expenses incurred under the agreements with FOKKISER are recorded as research and development expenses in the consolidated statements of operations and comprehensive income (loss). As of June 30, 2019, the Company had accrued \$0.2 million in expenses payable to FOKKISER under the agreement, which are included in accrued expenses and other current liabilities on the consolidated balance sheet.

## 12. Net Income (Loss) Per Share

The computations of basic and diluted net income (loss) per share are as follows (in thousands, except per share data):

|   | Three Months Ended June 30, |                | Six Months Ended June 30, |                |
|---|-----------------------------|----------------|---------------------------|----------------|
|   | 2019                        | 2018           | 2019                      | 2018           |
| Basic net income (loss) per share:                  |                             |                |                           |                |
| Net income (loss) applicable to common stockholders | \$ (1,457)                  | \$ 10,594      | \$ (33,685)               | \$ 114,833     |
| Shares used in computation:                         |                             |                |                           |                |
| Weighted-average common shares outstanding          | 36,669                      | 32,082         | 36,518                    | 31,858         |
| Basic net income (loss) per share                   | <u>\$ (0.04)</u>            | <u>\$ 0.33</u> | <u>\$ (0.92)</u>          | <u>\$ 3.60</u> |
| Diluted net income (loss) per share:                |                             |                |                           |                |
| Net income (loss) applicable to common stockholders | \$ (1,457)                  | \$ 10,594      | \$ (33,685)               | \$ 114,833     |
| Shares used in computation:                         |                             |                |                           |                |
| Weighted-average common shares outstanding          | 36,669                      | 32,082         | 36,518                    | 31,858         |
| Stock options                                       | —                           | 3,153          | —                         | 2,995          |
| Restricted stock units                              | —                           | 30             | —                         | 27             |
| Employee stock purchase plan                        | —                           | 7              | —                         | 4              |
| Weighted-average diluted common shares              | <u>36,669</u>               | <u>35,272</u>  | <u>36,518</u>             | <u>34,884</u>  |
| Diluted net income (loss) per share                 | <u>\$ (0.04)</u>            | <u>\$ 0.30</u> | <u>\$ (0.92)</u>          | <u>\$ 3.29</u> |

For periods in which the Company incurred net losses applicable to common stockholders, common stock equivalents are excluded from the calculation of diluted net loss per share as their effect would be anti-dilutive, and accordingly, basic and diluted net loss per share are the same for such periods. Outstanding stock options with exercise prices greater than the average market price of common stock are excluded from the calculation of diluted net income (loss) per share as their effect would be anti-dilutive. The following potentially dilutive common stock equivalents outstanding at the end of the period were excluded from the computations of weighted-average diluted common shares for the periods indicated as their effects would be anti-dilutive (in thousands):

|   | Three Months Ended June 30, |            | Six Months Ended June 30, |              |
|---|-----------------------------|------------|---------------------------|--------------|
|   | 2019                        | 2018       | 2019                      | 2018         |
| Stock options issued and outstanding        | 5,454                       | 804        | 5,454                     | 1,236        |
| Unvested restricted stock units outstanding | 40                          | —          | 40                        | —            |
| Employee stock purchase plan                | 26                          | —          | 26                        | —            |
|   | <u>5,520</u>                | <u>804</u> | <u>5,520</u>              | <u>1,236</u> |

**13. Supplemental Disclosures**

Accrued expenses and other current liabilities consists of the following (in thousands):

|  | <u>June 30, 2019</u> | <u>December 31, 2018</u> |
|--|----------------------|--------------------------|
| Accrued personnel costs                              | \$ 6,692             | \$ 9,484                 |
| Accrued external research and development expenses   | 4,925                | 4,274                    |
| Accrued sublicense fees and royalties                | 1,514                | 1,617                    |
| Accrued external general and administrative expenses | 1,498                | 773                      |
| Accrued income taxes payable                         | 564                  | 726                      |
| Accrued purchases of property and equipment          | 397                  | 221                      |
| Other accrued expenses and current liabilities       | 944                  | 69                       |
|  | <u>\$ 16,534</u>     | <u>\$ 17,164</u>         |

Other liabilities of \$1.9 million and \$2.5 million reported as of June 30, 2019 and December 31, 2018, respectively, consist of accrued sublicense fees payable to licensors in periods beyond 12 months from the reporting date.

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

You should read the following discussion and analysis of our financial condition and results of operations in conjunction with the financial statements and the notes thereto included elsewhere in this Quarterly Report on Form 10-Q and with our audited financial statements and the notes thereto included in our Annual Report on Form 10-K for the year ended December 31, 2018, which we filed with the SEC on February 27, 2019. In addition, you should read the "Risk Factors" and "Information Regarding Forward-Looking Statements" sections of this Quarterly Report on Form 10-Q and our Annual Report on Form 10-K for the year ended December 31, 2018 for a discussion of important factors that could cause actual results to differ materially from the results described in or implied by the forward-looking statements contained in the following discussion and analysis.

### Overview

We are a leading clinical-stage biotechnology company seeking to improve lives through the curative potential of gene therapy. Our gene therapy product candidates are designed to deliver genes to cells to address genetic defects or to enable cells in the body to produce therapeutic proteins that are intended to impact disease. Through a single administration, our gene therapy product candidates are designed to provide long-lasting effects, potentially significantly altering the course of disease and delivering improved patient outcomes.

### Overview of Product Candidates

We have developed an internal pipeline of product candidates, including ophthalmologic, central nervous system (CNS) and liver-directed therapies. Our therapeutic programs are comprised of both AAV-mediated antibodies for chronic diseases and gene therapy candidates for rare monogenic diseases.

#### *AAV-mediated antibody programs for the potential treatment of chronic diseases*

- **RGX-314:** We are developing RGX-314 for the treatment of wet age-related macular degeneration (wet AMD), a leading cause of blindness in the United States, Europe and Japan. On May 30, 2019, we announced completion of dosing in the Phase I/IIa clinical trial for RGX-314 for the treatment of wet AMD. Eight leading retinal surgery centers across the United States participated in the trial, designed to evaluate the safety and tolerability of RGX-314 in 42 dosed subjects across five escalating dose cohorts. Each subject received a single dose of RGX-314 administered by subretinal delivery. We expect to initiate a Phase IIb trial in late 2019. Additionally, we expect to file an investigational new drug (IND) application with the U.S. Food and Drug Administration (the FDA) for a Phase II trial to evaluate RGX-314 for the treatment of diabetic retinopathy in the second half of 2019.
- **AAV-Mediated Antibody Expression for the Treatment of Tauopathies:** Effective in July 2019, we entered into an exclusive license, development and commercialization agreement with Neurimmune AG (Neurimmune) to jointly develop and commercialize novel gene therapies using NAV Vectors to deliver human antibodies for chronic neurodegenerative diseases, with an initial focus on diseases associated with the accumulation and deposition of the microtubule-associated protein tau (tauopathies). The companies have initiated their first exclusive collaboration program for the treatment of tauopathies.
- **AAV-Mediated Antibody Expression for the Treatment of Hereditary Angioedema (HAE):** We are developing a novel, one-time treatment utilizing a NAV Vector to deliver a gene encoding for a therapeutic antibody that targets and binds to plasma kallikrein, a key protein left unregulated in patients with HAE. HAE is a chronic and severe disease characterized by recurring severe swelling (angioedema), most commonly in the face, airway, intestines and limbs.

#### *Gene therapy programs for the potential treatment of rare monogenic diseases*

- **RGX-501:** We are developing RGX-501 for the treatment of homozygous familial hypercholesterolemia (HoFH), a severe genetic disease characterized by premature and aggressive plaque buildup, life threatening coronary artery disease and aortic valve disease predominantly due to abnormalities in the function or expression of the low-density lipoprotein receptor. Dosing resumed and recruitment continues for the Phase I/II clinical trial for RGX-501 with corticosteroid prophylaxis.
- **RGX-121:** We are developing RGX-121 for the treatment of the neurological symptoms of Mucopolysaccharidosis Type II (MPS II), a severe genetic lysosomal storage disease caused by deficiency of iduronate-2-sulfatase (IDS), an enzyme that is responsible for breakdown of cellular waste products. Subject enrollment in the first of two dose cohorts continues in the Phase I/II clinical trial for RGX-121.

- **RGX-111:** We are developing RGX-111 for the treatment of the neurological symptoms of Mucopolysaccharidosis Type I (MPS I), a severe genetic lysosomal storage disease caused by deficiency of  $\alpha$ -L-iduronidase (IDUA), an enzyme required for breakdown of cellular waste products. Recruitment, screening and additional site activations are ongoing in the Phase I clinical trial for RGX-111.
- **RGX-181:** We are developing RGX-181 for the treatment of late-infantile neuronal ceroid lipofuscinosis type 2 (CLN2) disease, one of the most common forms of Batten disease, caused by mutations in the tripeptidyl peptidase 1 (TPP1) gene. We plan to file an IND application, or foreign equivalent, for RGX-181 for the treatment of CLN2 to the FDA, or a foreign regulatory authority, in the second half of 2019 to enable initiation of a first-in-human clinical trial.

In addition to our lead product candidates described above, we have also funded, and plan to continue to fund, preclinical research on potential product candidate programs that may become part of our internal product development pipeline. We have partnered with a number of leading academic institutions and will continue to seek partnerships with innovative institutions to develop novel NAV gene therapy product candidates.

### ***RGX-314 Interim Data Update***

In our Phase I/IIa trial for RGX-314, as of July 31, 2019, 42 subjects with wet AMD have received a single administration of RGX-314 across five dose cohorts. To qualify for inclusion in the trial, participants were required to have a history of frequent anti-vascular endothelial growth factor (VEGF) treatments (including at least four anti-VEGF injections in the eight months preceding trial enrollment) and a documented history of response to anti-VEGF therapy. The trial design included doses of  $3 \times 10^9$  (Cohort 1),  $1 \times 10^{10}$  (Cohort 2),  $6 \times 10^{10}$  (Cohort 3),  $1.6 \times 10^{11}$  (Cohort 4) and  $2.5 \times 10^{11}$  (Cohort 5) genome copies (GC)/eye. Subjects will be assessed every four weeks to the six-month primary endpoint, with long-term follow-up continuing for two years. Below is a summary of the preliminary results of our Phase I/IIa trial as of July 31, 2019:

- RGX-314 continues to be well-tolerated across all five dose cohorts, with no drug related serious adverse events (SAEs) reported.
- Dose-dependent increases in RGX-314 protein expression levels, as measured from aqueous samples by electrochemiluminescence immunoassay (ECL) at approximately one month after administration of RGX-314, have been observed across all doses.
- 50% of subjects (3/6) in Cohort 3 continue to remain injection-free at 18 months.

### ***Overview of Our NAV Technology Platform***

In addition to our internal product development efforts, we also selectively license our proprietary adeno-associated virus (AAV) gene therapy delivery platform (NAV Technology Platform) to other leading biotechnology and pharmaceutical companies, which we refer to as NAV Technology Licensees. As of June 30, 2019, our NAV Technology Platform was being applied in the development of more than 20 partnered product candidates by our NAV Technology Licensees. Licensing the NAV Technology Platform allows us to maintain our internal product development focus on our core disease indications and therapeutic areas while still expanding the NAV gene therapy pipeline, developing a greater breadth of treatments for patients, providing additional technological and potential clinical proof-of-concept for our NAV Technology Platform, and creating potential additional revenue.

### **Financial Overview**

#### ***Revenues***

Our revenues to date primarily consist of license and royalty revenue resulting from the licensing of our NAV Technology Platform. We have not generated any revenues from commercial sales of our own products. If we fail to complete the development of our product candidates in a timely manner or fail to obtain their regulatory approval and adequate labeling, our ability to generate future revenues will be materially compromised.

We license our NAV Technology Platform to other biotechnology and pharmaceutical companies. As of June 30, 2019, our NAV Technology Platform was being applied in the development of more than 20 partnered product candidates by nine NAV Technology Licensees. The terms of the licenses vary, and licenses may be exclusive or non-exclusive and may be sublicensable by the licensee. Licenses may grant intellectual property rights for purposes of internal and preclinical research and development only, or may include the rights, or options to obtain future rights, to commercialize drug therapies for specific diseases using the NAV Technology Platform. License agreements generally have a term at least equal to the life of the underlying patents, but are terminable at the option of the licensee. Consideration from licensees under our license agreements may include: (i) up-front and annual fees, (ii) option fees to acquire additional licenses, (iii) milestone payments based on the achievement of certain development and sales-based milestones by licensees, (iv) sublicense fees and (v) royalties on sales of licensed products.

Royalty revenue to date consists of royalties on net sales of Zolgensma®, which is marketed by AveXis, Inc. (AveXis), a wholly owned subsidiary of Novartis AG (Novartis). Zolgensma is a licensed product under our March 2014 license agreement, as amended, with AveXis for the development and commercialization of treatments for spinal muscular atrophy (SMA).

Future license and royalty revenues are dependent on the successful development and commercialization of licensed products by our licensees, which is uncertain, and revenue may fluctuate significantly from period to period. Additionally, we may never receive consideration in our license agreements that is contemplated on option fees, development and sales-based milestone payments, royalties on sales of licensed products or sublicense fees, given the contingent nature of these payments. Our revenues are concentrated among a low number of licensees and licenses are terminable at the option of the licensee. The termination of our licenses by licensees may materially impact the amount of revenue we recognize in future periods.

### *Operating Expenses*

Our operating expenses consist primarily of cost of revenues, research and development expenses and general and administrative expenses. Personnel costs including salaries, benefits, bonuses and stock-based compensation expense, comprise a significant component of research and development and general and administrative expenses. We allocate indirect expenses associated with our facilities, information technology costs, depreciation and other overhead costs between research and development and general and administrative categories based on employee headcount and the nature of work performed by each employee.

#### *Cost of Revenues*

Our cost of revenues consists primarily of upstream fees due to our licensors as a result of revenue generated from the licensing of our NAV Technology Platform, including sublicense fees, milestone payments and royalties on net sales of licensed products. Sublicense fees are based on a percentage of license fees received by us from NAV Technology Licensees and are recognized in the period that the underlying license revenue is recognized. Milestone payments are payable to licensors upon the achievement of specified milestones by NAV Technology Licensees and are recognized in the period the milestone is achieved or deemed probable of achievement. Royalties are based on a percentage of net sales of licensed products by NAV Technology Licensees and are recognized in the period that the underlying sales occur. Future costs of revenues are uncertain due to the nature of our license agreements and significant fluctuations in cost of revenues may occur from period to period.

#### *Research and Development Expense*

Our research and development expense primarily consists of:

- salaries and personnel-related costs, including benefits, stock-based compensation and travel, for our scientific personnel performing research and development activities;
- costs related to executing preclinical studies and clinical trials;
- costs related to acquiring, developing and manufacturing materials for preclinical studies and clinical trials;
- fees paid to consultants and other third-parties who support our product candidate development;
- other costs in seeking regulatory approval of our product candidates; and
- allocated facility-related costs, depreciation expense and other overhead.

Up-front fees incurred in obtaining technology licenses for research and development activities are expensed as incurred if the technology licensed has no alternative future use.

We plan to increase our research and development expenses for the foreseeable future as we continue development of our product candidates. Our current and planned research and development activities include the following:

- a Phase I/IIa clinical trial and a planned Phase IIb clinical trial to evaluate the safety and efficacy of our RGX-314 program for the treatment of wet AMD, and a planned Phase II clinical trial to evaluate the safety and efficacy of our RGX-314 program for the treatment of diabetic retinopathy;
- a Phase I/II clinical trial to evaluate the safety and efficacy of our RGX-501 program for the treatment of HoFH;
- a Phase I/II clinical trial to evaluate the safety and efficacy of our RGX-121 program for the treatment of MPS II;
- a Phase I clinical trial to evaluate the safety and efficacy of our RGX-111 program for the treatment of MPS I;

- preclinical research and development and a planned clinical trial for our RGX-181 program for the treatment of CLN2;
- preclinical research and development for potential product candidates to treat neurodegenerative diseases, including tauopathies, under our collaboration with Neurimmune;
- preclinical research and development for potential product candidates to treat HAE;
- preclinical research and development for additional product candidates addressing other diseases in the ophthalmologic, CNS and liver-directed therapeutic areas;
- continued investment in advanced manufacturing analytics and process development activities; and
- continued acquisition and manufacture of clinical trial materials in support of our anticipated clinical trials.

The following table summarizes our research and development expenses incurred during the three and six months ended June 30, 2019 and 2018 (in thousands):

|                                     | Three Months Ended June 30, |           | Six Months Ended June 30, |           |
|-------------------------------------|-----------------------------|-----------|---------------------------|-----------|
|                                     | 2019                        | 2018      | 2019                      | 2018      |
| <b>Direct Expenses</b>              |                             |           |                           |           |
| RGX-314                             | \$ 3,582                    | \$ 1,528  | \$ 5,986                  | \$ 3,148  |
| RGX-501                             | 1,066                       | 4,889     | 2,065                     | 9,038     |
| RGX-121                             | 299                         | 832       | 2,073                     | 1,791     |
| RGX-111                             | 767                         | 920       | 1,608                     | 2,153     |
| RGX-181                             | 3,007                       | —         | 5,303                     | —         |
| Total direct expenses               | 8,721                       | 8,169     | 17,035                    | 16,130    |
| <b>Unallocated Expenses</b>         |                             |           |                           |           |
| Unallocated external expenses       | 5,459                       | 3,037     | 8,855                     | 5,653     |
| Personnel-related                   | 12,241                      | 8,409     | 22,815                    | 15,780    |
| Facilities and depreciation expense | 2,290                       | 1,339     | 4,496                     | 2,561     |
| Other unallocated                   | 772                         | 532       | 1,485                     | 912       |
| Total unallocated expenses          | 20,762                      | 13,317    | 37,651                    | 24,906    |
| Total research and development      | \$ 29,483                   | \$ 21,486 | \$ 54,686                 | \$ 41,036 |

Expenses incurred in the development of RGX-181 were included in unallocated external expenses through the second quarter of 2018. Unallocated external expenses include direct costs not identifiable with a specific lead product candidate, including costs associated with our research and development platform, process development, manufacturing analytics and preclinical research and development for prospective product candidates and new technologies. We typically utilize our employee and infrastructure resources across our development programs. We do not allocate personnel and other internal costs, such as facilities and other overhead costs, to specific product candidates or development programs.

#### *General and Administrative Expense*

Our general and administrative expense consists primarily of salaries and personnel-related costs, including employee travel, benefits and stock-based compensation, for employees performing functions other than research and development. This includes certain personnel in executive, commercial, corporate development, finance, legal, human resources, information technology and administrative support functions. Other general and administrative expenses include facility-related and overhead costs not otherwise allocated to research and development expense, professional fees for accounting, legal and advisory services, expenses associated with obtaining and maintaining patents, insurance costs, costs of our information systems and other commercial and general corporate activities. We expect that our general and administrative expense will continue to increase as we continue to develop, and potentially commercialize, our product candidates.

## **Other Income**

### *Interest Income from Licensing*

In accordance with our revenue recognition policy, interest income from licensing consists of imputed interest recognized from significant financing components identified in our license agreements with NAV Technology Licensees.

### *Investment Income*

Investment income consists of interest income earned and gains and losses realized from our cash equivalents and marketable securities, as well as unrealized gains and losses on marketable equity securities. Cash equivalents are comprised of money market mutual funds and highly liquid debt securities with original maturities of 90 days or less at acquisition. Marketable securities are comprised of available-for-sale debt securities and equity securities.

## **Critical Accounting Policies and Significant Judgments and Estimates**

This Management's Discussion and Analysis of Financial Condition and Results of Operations is based on our financial statements, which we have prepared in accordance with accounting principles generally accepted in the United States. The preparation of our financial statements requires us to make estimates and assumptions that affect the reported amounts of assets and liabilities and the disclosure of contingent assets and liabilities at the date of our financial statements, as well as the reported revenues and expenses during the reported periods. We evaluate these estimates and assumptions on an ongoing basis. We base our estimates on historical experience and on various other factors that we believe are reasonable under the circumstances, the results of which form the basis for making judgments about the carrying value of assets and liabilities that are not readily apparent from other sources. Actual results may differ from these estimates under different assumptions or conditions.

Our significant accounting policies and recently announced accounting pronouncements, including the expected impact of such pronouncements, are fully described in Note 2 to the accompanying unaudited consolidated financial statements and in Note 2 to our audited consolidated financial statements which are included in our Annual Report on Form 10-K for the year ended December 31, 2018. Other than the critical accounting policies discussed below, there have been no significant changes in our critical accounting policies since December 31, 2018.

## **Revenue Recognition**

We recognize revenue in accordance with Accounting Standards Codification (ASC) 606, *Revenue from Contracts with Customers* (Topic 606). Topic 606 requires entities to recognize revenue when control of the promised goods or services is transferred to customers at an amount that reflects the consideration to which the entity expects to be entitled to in exchange for those goods or services. The following five steps are performed to determine the appropriate revenue recognition for arrangements within the scope of Topic 606: (i) identify the contract(s) with a customer, (ii) identify the performance obligations in the contract, (iii) determine the transaction price, (iv) allocate the transaction price to the performance obligations in the contract and (v) recognize revenue when (or as) the entity satisfies the performance obligations.

We apply the five-step model to contracts that are within the scope of Topic 606 only when it is probable that we will collect the consideration we are entitled to in exchange for the goods or services we transfer to the customer. At contract inception, for contracts within the scope of Topic 606, we assess the goods or services promised within each contract and determine those that are performance obligations and whether each promised good or service is distinct. We then recognize as revenue the amount of the transaction price that is allocated to respective performance obligations when (or as) the respective performance obligations are satisfied.

We evaluate our contracts with customers for the presence of significant financing components. If a significant financing component is identified in a contract and provides a financing benefit to the customer, the transaction price for the contract is adjusted to account for the financing portion of the arrangement, which is recognized as interest income over the financing term using the effective interest method. In determining the appropriate interest rates for significant financing components, we evaluate the credit profile of the customer and prevailing market interest rates and select an interest rate in which we believe would be charged to the customer in a separate financing arrangement over a similar financing term.

### *License and Royalty Revenue*

We license our NAV Technology Platform to other biotechnology and pharmaceutical companies. The terms of the licenses vary, and licenses may be exclusive or non-exclusive and may be sublicensable by the licensee. Licenses may grant intellectual property rights for purposes of internal and preclinical research and development only, or may include the rights, or options to obtain future rights, to commercialize drug therapies for specific diseases using our NAV Technology Platform. License agreements generally have a term at least equal to the life of the underlying patents, but are terminable at the option of the licensee. Consideration payable to us under our license agreements may include: (i) up-front and annual fees, (ii) option fees to acquire additional licenses, (iii) milestone payments based on the achievement of certain development and sales-based milestones by licensees, (iv) sublicense fees and (v) royalties on sales of licensed products.

Our license agreements are accounted for as contracts with customers within the scope of Topic 606. At the inception of each license agreement, we determine the contract term for purposes of applying the requirements of Topic 606. Licenses are generally terminable at the option of the licensee with advance notice to us. For each license, we evaluate these termination rights to determine whether a substantive termination penalty would be incurred by the licensee upon termination. If the licensee incurs a substantive termination penalty upon termination, the contract term for revenue recognition purposes is generally equal to the stated term of the license, which is the life of the underlying licensed patents. Alternatively, if the licensee does not incur a substantive termination penalty upon termination, the contract term for revenue recognition purposes may be shorter than the stated term of the license, in which case the termination rights may be accounted for as contract renewal options. The determination of whether a substantive termination penalty is associated with the termination rights requires significant judgment. In making this determination, we consider, among other things, the nature of the intellectual property rights that would be returned to us upon termination, including the exclusivity of the licensed rights and the stage of development of the licensed products, the payment terms, including the amount and timing of non-refundable or guaranteed payments, and the business purpose of the termination rights granted to the licensee. We consider all of the facts and circumstances relevant to each license when making this determination.

Performance obligations under our license agreements may include (i) the delivery of intellectual property licenses and (ii) options granted to licensees to acquire additional licenses to the extent the options represent material rights to the licensee. At the inception of each license agreement which contains options for the licensee to acquire additional licenses, or contract renewal options, we evaluate the options to determine whether they provide material rights to the licensee. In making this determination, we consider whether the options are priced at a discount to the standalone selling price for the underlying licenses. If an option is priced at a discount to the standalone selling price for the underlying license, the option is considered to be a material right to the licensee and is accounted for as a separate performance obligation under the current license agreement.

We evaluate the transaction price of our license agreements at the inception of each agreement and at each reporting date. The transaction price includes the fixed consideration payable to us during the contract term, as well as any variable consideration to the extent that it is probable that a significant reversal of revenue will not occur in the future. Fixed consideration under the license agreements includes up-front and annual fees payable during the contract term. Variable consideration under the license agreements includes development and sales-based milestone payments, sublicense fees and royalties on sales of licensed products. Consideration contingent upon the exercise of options by a licensee is excluded from the transaction price and not accounted for as part of the license agreement until the option is exercised.

The transaction price for each license agreement is allocated to the underlying performance obligations and recognized as revenue when the performance obligations are satisfied. Consideration allocated to performance obligations for the delivery of an intellectual property license is recognized as revenue in full upon the delivery of the license to the licensee. Consideration allocated to performance obligations for license options is recognized as revenue in full upon the earlier of the option exercise or expiration. The exercise of a license option by a licensee is accounted for as a new license for revenue recognition purposes.

Up-front and annual license fees payable to us over the contract term of each license are included in the transaction price, and the portion of this consideration that is allocated to the performance obligation for the delivery of the intellectual property license is recognized as revenue in full upon the delivery of the license to the licensee. If annual license fees are payable to us in periods beyond 12 months from the delivery of the license, a significant financing component is deemed to exist which provides a financing benefit to the licensee. If a significant financing component is identified, we adjust the transaction price for the license to include only the present value of the annual license fees payable to us over the contract term. The discounted portion of the license fees is recognized as interest income from licensing over the financing period of the license.

Development milestone payments are payable to us upon the achievement of specified development milestones by licensees. At the inception of each license agreement that contains development milestone payments, we evaluate whether the milestones are considered probable of achievement and estimate the amount to be included in the transaction price using the most likely amount method. If it is probable that a significant revenue reversal will not occur in the future, milestone payments are included in the transaction price and recognized as revenue upon the delivery of the license. Milestone payments contingent on the achievement of development milestones that are not within our control or the control of the licensee, such as regulatory approvals, are not considered probable of being achieved and are excluded from the transaction price until the milestone is achieved. At each reporting date, we re-evaluate the probability of achievement of outstanding development milestones and, if necessary, adjust the transaction price for any milestones for which the probability of achievement has changed due to current facts and circumstances. Any such adjustments are recorded on a cumulative catch-up basis and recognized as revenue in the period of the adjustment.

Royalties on sales of licensed products, sales-based milestone payments and sublicense fees based on the receipt of certain fees by licensees from any sublicensees are excluded from the transaction price of each license and recognized as revenue in the period that the related sales or sublicenses occur, provided that the associated license has been delivered to the licensee.

Royalty revenue to date consists of royalties on net sales of Zolgensma, which is marketed by AveXis, a wholly owned subsidiary of Novartis. Zolgensma is a licensed product under our March 2014 license agreement, as amended, with AveXis for the development and commercialization of treatments for SMA. We recognize royalty revenue from net sales of Zolgensma in the period in which the underlying products are sold by AveXis, which in certain cases may require us to estimate royalty revenue for periods of net sales which have not yet been reported to us. Sales-based milestone payments related to net sales of Zolgensma are recognized as royalty revenue in the period in which the milestone is achieved.

We receive payments from licensees based on the billing schedules established in each license agreement. Amounts recognized as revenue which have not yet been received from licensees, including unbilled royalties, are recorded as accounts receivable when our rights to the consideration are conditional solely upon the passage of time. Amounts recognized as revenue which have not yet been received from licensees are recorded as contract assets when our rights to the consideration are not unconditional. Contract assets are recorded as other current assets on the consolidated balance sheets. If a licensee elects to terminate a license prior to the end of the license term, the licensed intellectual property is returned to us and any consideration recorded as accounts receivable or contract assets which is not contractually payable by the licensee is charged off as a reduction of license revenue in the period of the termination. Amounts received by us prior to the delivery of underlying performance obligations are deferred and recognized as revenue upon the satisfaction of the performance obligations. Deferred revenue which is not expected to be recognized within 12 months from the reporting date is recorded as non-current on the consolidated balance sheets.

#### **Recent Accounting Pronouncements**

See Note 2 “Recent Accounting Pronouncements” in the notes to the accompanying unaudited consolidated financial statements for a full description of recently announced accounting pronouncements and the expected impact to our financial statements.

In February 2016, the Financial Accounting Standards Board (FASB) issued Accounting Standards Update (ASU) 2016-02, *Leases* (Topic 842) which supersedes the lease accounting requirements in ASC 840, *Leases* (Topic 840). Effective January 1, 2019, we adopted Topic 842 using the modified retrospective transition method. Under this method, we applied Topic 842 to all leases in effect as of, or entered into after, January 1, 2019 and recorded the cumulative impact of the adoption as an adjustment to our accumulated deficit on January 1, 2019. Our consolidated financial statements for periods ending after January 1, 2019 are presented in accordance with the requirements of Topic 842, while comparative prior period amounts have not been adjusted and continue to be reported in accordance with Topic 840.

Upon the adoption of Topic 842, we recorded operating lease right-of-use assets of \$7.4 million and operating lease liabilities of \$8.4 million for our leases which were in effect and had commenced prior to January 1, 2019 and had original lease terms of more than 12 months. We also derecognized current and non-current deferred rent liabilities of \$1.4 million and prepaid expenses, other current assets and other assets of \$0.4 million upon the adoption of Topic 842. Additionally, upon the adoption of Topic 842, we derecognized \$5.9 million of property and equipment and \$5.9 million of financing lease obligations related to construction-in-progress at 9800 Medical Center Drive, as we do not control the building during the construction period under the requirements of Topic 842. The cumulative impact of the adoption of Topic 842 resulted in an increase in accumulated deficit of less than \$0.1 million on January 1, 2019. The adoption of Topic 842 did not have a material impact on our results of operations for the three and six months ended June 30, 2019, nor do we believe it will have a material impact on future results of operations based on our current leasing arrangements.

## Results of Operations

|                                     | Three Months Ended June 30, |           |             | Six Months Ended June 30, |            |              |
|-------------------------------------|-----------------------------|-----------|-------------|---------------------------|------------|--------------|
|                                     | 2019                        | 2018      | Change      | 2019                      | 2018       | Change       |
| (in thousands)                      |                             |           |             |                           |            |              |
| <b>Revenues</b>                     |                             |           |             |                           |            |              |
| License and royalty revenue         | \$ 7,881                    | \$ 40,031 | \$ (32,150) | \$ 8,765                  | \$ 172,422 | \$ (163,657) |
| Total revenues                      | 7,881                       | 40,031    | (32,150)    | 8,765                     | 172,422    | (163,657)    |
| <b>Operating Expenses</b>           |                             |           |             |                           |            |              |
| Cost of revenues                    | 1,927                       | 3,872     | (1,945)     | 1,956                     | 6,280      | (4,324)      |
| Research and development            | 29,483                      | 21,486    | 7,997       | 54,686                    | 41,036     | 13,650       |
| General and administrative          | 13,405                      | 8,318     | 5,087       | 24,963                    | 16,698     | 8,265        |
| Other operating expenses (income)   | (62)                        | 5         | (67)        | (62)                      | 33         | (95)         |
| Total operating expenses            | 44,753                      | 33,681    | 11,072      | 81,543                    | 64,047     | 17,496       |
| Income (loss) from operations       | (36,872)                    | 6,350     | (43,222)    | (72,778)                  | 108,375    | (181,153)    |
| <b>Other Income</b>                 |                             |           |             |                           |            |              |
| Interest income from licensing      | 762                         | 6,898     | (6,136)     | 1,375                     | 8,253      | (6,878)      |
| Investment income                   | 34,524                      | 1,196     | 33,328      | 37,519                    | 2,055      | 35,464       |
| Total other income                  | 35,286                      | 8,094     | 27,192      | 38,894                    | 10,308     | 28,586       |
| Income (loss) before income taxes   | (1,586)                     | 14,444    | (16,030)    | (33,884)                  | 118,683    | (152,567)    |
| <b>Income Tax Benefit (Expense)</b> |                             |           |             |                           |            |              |
|                                     | 129                         | (3,850)   | 3,979       | 199                       | (3,850)    | 4,049        |
| Net income (loss)                   | \$ (1,457)                  | \$ 10,594 | \$ (12,051) | \$ (33,685)               | \$ 114,833 | \$ (148,518) |

### Comparison of the Three Months Ended June 30, 2019 and 2018

**License and Royalty Revenue.** License and royalty revenue decreased by \$32.2 million, from \$40.0 million for the three months ended June 30, 2018 to \$7.9 million for the three months ended June 30, 2019. The decrease was primarily attributable to \$40.0 million of non-recurring license revenue recognized during the three months ended June 30, 2018 under our amended March 2014 license agreement with AveXis. In May 2018, AveXis was acquired by Novartis, which triggered the acceleration of certain payments under the license agreement including \$40.0 million which was previously payable upon the achievement of a specified sales-based milestone. Upon the completion of the acquisition, we recognized \$40.0 million of license revenue related to the accelerated sales-based milestone payment, which had previously been excluded from the transaction price for the license agreement. The decrease was partially offset by revenue recognized during the three months ended June 30, 2019 resulting from license options exercised and development milestones achieved by licensees during the period, as well as royalties on net sales of Zolgensma. Commercial sales of Zolgensma commenced in the second quarter of 2019, upon which we began recognizing royalty revenue from net sales of the licensed product. We expect royalties to increase in future periods. We are also eligible to receive a milestone payment of \$80.0 million from AveXis upon the achievement of \$1.0 billion in cumulative net sales of Zolgensma. The decrease in revenue during the three months ended June 30, 2019 resulted in a decrease in cost of revenues incurred during the period, primarily related to sublicense fees that we are obligated to pay to our licensors.

**Research and Development Expense.** Research and development expenses increased by \$8.0 million, from \$21.5 million for the three months ended June 30, 2018 to \$29.5 million for the three months ended June 30, 2019. The increase was primarily attributable to the following:

- an increase of \$3.8 million for personnel costs as a result of increased headcount of research and development personnel, including a \$1.6 million increase in stock-based compensation expense;
- an increase of \$1.9 million for external costs associated with clinical trial activities for our lead product candidates and manufacturing-related services; and
- an increase of \$1.3 million for laboratory costs and facilities used by research and development personnel, including a \$0.8 million increase in depreciation expense allocated to research and development functions.

*General and Administrative Expense.* General and administrative expenses increased by \$5.1 million, from \$8.3 million for the three months ended June 30, 2018 to \$13.4 million for the three months ended June 30, 2019. The increase was primarily attributable to the following:

- an increase of \$2.6 million for personnel costs as a result of increased headcount of general and administrative personnel, including a \$1.5 million increase in stock-based compensation expense;
- an increase of \$1.1 million for professional services, including commercial, legal, accounting and other advisory services; and
- an increase of \$0.8 million for costs related to the maintenance of patents licensed from third parties.

*Interest Income from Licensing.* Interest income from licensing decreased by \$6.1 million, from \$6.9 million for the three months ended June 30, 2018 to \$0.8 million for the three months ended June 30, 2019. The decrease was primarily attributable to \$6.8 million of interest income recognized during the three months ended June 30, 2018 under our amended March 2014 license agreement with AveXis, \$6.1 million of which was recognized upon the acceleration of license payments triggered by the acquisition of AveXis by Novartis in May 2018.

*Investment Income.* Investment income increased by \$33.3 million, from \$1.2 million for the three months ended June 30, 2018 to \$34.5 million for the three months ended June 30, 2019. The increase was primarily attributable to an unrealized gain of \$31.7 million recognized during the three months ended June 30, 2019 related to our marketable equity securities of Prevail Therapeutics Inc. (Prevail). We acquired the securities as consideration for a commercial license to the NAV Technology Platform granted to Prevail in August 2017. Prevail completed its initial public offering (IPO) in June 2019. Prior to Prevail's IPO, the securities were accounted for as non-marketable equity securities without a readily determinable fair value and had a carrying value of \$0.4 million. Upon Prevail's IPO in June 2019, the securities were reclassified to marketable securities and are measured at fair value. Pursuant to a lock-up agreement executed in connection with Prevail's IPO, we are restricted from selling our common stock of Prevail prior to December 2019. As of June 30, 2019, no amounts have been realized by us related to our marketable equity securities of Prevail as there have been no sales of the securities. Significant fluctuations in the fair value of the securities may occur from period to period.

### **Comparison of the Six Months Ended June 30, 2019 and 2018**

*License and Royalty Revenue.* License and royalty revenue decreased by \$163.7 million, from \$172.4 million for the six months ended June 30, 2018 to \$8.8 million for the six months ended June 30, 2019. The decrease was primarily attributable to \$172.1 million of non-recurring license revenue recognized during the six months ended June 30, 2018 under our amended March 2014 license agreement with AveXis. The decrease was partially offset by revenue recognized during the six months ended June 30, 2019 resulting from license options exercised and development milestones achieved by licensees during the period, as well as royalties on net sales of Zolgensma. Commercial sales of Zolgensma commenced in the second quarter of 2019, upon which we began recognizing royalty revenue from net sales of the licensed product. We expect royalties to increase in future periods. We are also eligible to receive a milestone payment of \$80.0 million from AveXis upon the achievement of \$1.0 billion in cumulative net sales of Zolgensma. The decrease in revenue during the six months ended June 30, 2019 resulted in a decrease in cost of revenues incurred during the period, primarily related to sublicense fees that we are obligated to pay to our licensors.

*Research and Development Expense.* Research and development expenses increased by \$13.7 million, from \$41.0 million for the six months ended June 30, 2018 to \$54.7 million for the six months ended June 30, 2019. The increase was primarily attributable to the following:

- an increase of \$7.0 million for personnel costs as a result of increased headcount of research and development personnel, including a \$2.4 million increase in stock-based compensation expense;
- an increase of \$2.8 million for laboratory costs and facilities used by research and development personnel, including a \$1.6 million increase in depreciation expense allocated to research and development functions; and
- an increase of \$2.2 million for external costs associated with clinical trial activities for our lead product candidates and manufacturing-related services.

*General and Administrative Expense.* General and administrative expenses increased by \$8.3 million, from \$16.7 million for the six months ended June 30, 2018 to \$25.0 million for the six months ended June 30, 2019. The increase was primarily attributable to the following:

- an increase of \$4.6 million for personnel costs as a result of increased headcount of general and administrative personnel, including a \$3.1 million increase in stock-based compensation expense;
- an increase of \$2.1 million for professional services, including commercial, legal, accounting and other advisory services; and
- an increase of \$0.4 million for costs related to the maintenance of patents licensed from third parties.

*Interest Income from Licensing.* Interest income from licensing decreased by \$6.9 million, from \$8.3 million for the six months ended June 30, 2018 to \$1.4 million for the six months ended June 30, 2019. The decrease was primarily attributable to \$8.0 million of interest income recognized during the six months ended June 30, 2018 under our amended March 2014 license agreement with AveXis, \$6.1 million of which was recognized upon the acceleration of license payments triggered by the acquisition of AveXis by Novartis in May 2018.

*Investment Income.* Investment income increased by \$35.5 million, from \$2.1 million for the six months ended June 30, 2018 to \$37.5 million for the six months ended June 30, 2019. The increase was primarily attributable to an unrealized gain of \$31.7 million recognized during the six months ended June 30, 2019 related to our marketable equity securities of Prevail. We acquired the securities as consideration for a commercial license to the NAV Technology Platform granted to Prevail in August 2017. Prevail completed its IPO in June 2019. Prior to Prevail's IPO, the securities were accounted for as non-marketable equity securities without a readily determinable fair value and had a carrying value of \$0.4 million. Upon Prevail's IPO in June 2019, the securities were reclassified to marketable securities and are measured at fair value. Pursuant to a lock-up agreement executed in connection with Prevail's IPO, we are restricted from selling our common stock of Prevail prior to December 2019. As of June 30, 2019, no amounts have been realized by us related to our marketable equity securities of Prevail as there have been no sales of the securities. Significant fluctuations in the fair value of the securities may occur from period to period.

## **Liquidity and Capital Resources**

As of June 30, 2019, we had cash, cash equivalents and marketable securities of \$449.7 million, which were primarily derived from the sale of common stock as well as revenues generated from the licensing of our NAV Technology Platform. We expect that our cash, cash equivalents and marketable securities as of June 30, 2019, will enable us to fund our operating expenses and capital expenditure requirements for at least the next 12 months from the date of this report, based on our current business plan.

Commercial sales of Zolgensma commenced in the second quarter of 2019, upon which we began recognizing royalty revenue on net sales of the licensed product. We are also eligible to receive, in addition to other development milestone payments, a milestone payment of \$80.0 million from AveXis upon the achievement of \$1.0 billion in cumulative net sales of Zolgensma.

During the six months ended June 30, 2019, we recognized an unrealized gain of \$31.7 million related to our marketable equity securities of Prevail, which completed its IPO in June 2019. We acquired the securities as consideration for a commercial license to the NAV Technology Platform granted to Prevail in August 2017. As of June 30, 2019, our marketable equity securities of Prevail had a fair value of \$32.1 million. We are restricted from selling our common stock of Prevail prior to December 2019, and significant fluctuations in the fair value of the securities may occur from period to period.

We have incurred cumulative losses since our inception and had an accumulated deficit of \$116.8 million as of June 30, 2019. Our transition to recurring profitability is dependent upon the successful development, approval and commercialization of our product candidates and achieving a level of revenues adequate to support our cost structure. We do not expect to achieve such revenues, and expect to continue to incur losses, for at least the next several years. We expect that our research and development and general and administrative expenses will continue to increase for the foreseeable future as we continue the development of, and seek regulatory approval for, our product candidates. Subject to obtaining regulatory approval for our product candidates, we expect to incur significant commercialization expenses for product sales, marketing, manufacturing and distribution. Additionally, we expect our capital expenditures will increase significantly in the future for costs associated with building out additional office, laboratory and manufacturing capacity to further support the development of our product candidates and potential commercialization efforts. As a result, we will need significant additional capital to fund our operations, which we may obtain through one or more equity offerings, debt financings or other third-party funding, including potential strategic alliances and licensing or collaboration arrangements.

**Cash Flows**

|  | <b>Six Months Ended June 30,</b> |                  |
|--|----------------------------------|------------------|
|  | <b>2019</b>                      | <b>2018</b>      |
|  | (in thousands)                   |                  |
| Net cash provided by (used in) operating activities                      | \$ (53,046)                      | \$ 128,345       |
| Net cash provided by (used in) investing activities                      | 27,133                           | (75,453)         |
| Net cash provided by financing activities                                | 5,494                            | 7,341            |
| Net increase (decrease) in cash and cash equivalents and restricted cash | <u>\$ (20,419)</u>               | <u>\$ 60,233</u> |

*Operating Activities*

Our net cash used in operating activities for the six months ended June 30, 2019 increased by \$181.4 million from the six months ended June 30, 2018. The change was primarily attributable to \$180.0 million in license payments we received during the six months ended June 30, 2018 related to the amendment of our March 2014 license agreement with AveXis, as well as an increase in operating expenses in the six months ended June 30, 2019. The increase in operating expenses was primarily attributable to increased employee headcount and external research and development expenses as we continue the development and advancement of our lead product candidates.

For the six months ended June 30, 2019, our net cash used in operating activities of \$53.0 million consisted of a net loss of \$33.7 million, \$17.2 million in adjustments for non-cash items and changes in working capital of \$2.2 million. Adjustments for non-cash items primarily consisted of an unrealized gain on our marketable equity securities of Prevail of \$31.7 million, imputed interest earned from our license agreements of \$1.4 million and net accretion of discounts on marketable debt securities of \$0.8 million, and were partially offset by stock-based compensation expenses of \$12.8 million and depreciation and amortization expense of \$3.4 million. The change in working capital was primarily attributable to an increase in accounts receivable of \$1.4 million, an increase in other assets of \$1.4 million, a decrease in accrued expenses and other current liabilities of \$1.0 million, a decrease in deferred revenue of \$0.6 million and an increase in prepaid expenses of \$0.5 million, and was partially offset by an increase in accounts payable of \$1.8 million and a decrease in other current assets of \$1.4 million. The increase in accounts receivable and decrease in deferred revenue were largely driven by new licenses we granted as a result of license options exercised by licensees during the period. The increases in prepaid expenses and other assets were largely driven by an increase in the amounts we were billed by service providers at the end of the period which applicable to future periods of performance, including periods beyond 12 months from June 30, 2019.

For the six months ended June 30, 2018, our net cash provided by operating activities of \$128.3 million consisted of net income of \$114.8 million, \$1.5 million in adjustments for non-cash items and changes in working capital of \$12.0 million. Adjustments for non-cash items primarily consisted of stock-based compensation expenses of \$7.3 million, depreciation and amortization expense of \$1.7 million and net amortization of premiums on marketable debt securities of \$0.7 million and were partially offset by imputed interest earned from our license agreements of \$8.3 million. The change in working capital was primarily attributable to a decrease in accounts receivable of \$8.9 million, an increase in accrued expenses and other current liabilities of \$2.6 million and a decrease in prepaid expenses of \$1.6 million. The decrease in accounts receivable was largely driven by the imputed interest recognized upon the acceleration of license payments under our amended license agreement with AveXis. The increase in accrued expenses and other current liabilities was largely driven by an increase in income taxes payable as of June 30, 2018 as a result of income generated during the period. The decrease in prepaid expenses was largely driven by a decrease in the amounts we were billed for at the end of the period which are applicable to future periods of performance for preclinical, manufacturing and clinical trial activities.

*Investing Activities*

For the six months ended June 30, 2019, net cash provided by investing activities consisted of \$141.3 million in sales and maturities of marketable securities, offset by \$106.1 million to purchase marketable securities and \$8.0 million to purchase property and equipment.

For the six months ended June 30, 2018, net cash used in investing activities consisted of \$139.1 million to purchase marketable securities and \$5.0 million to purchase property and equipment, offset by \$68.6 million in sales and maturities of marketable securities.

### *Financing Activities*

For the six months ended June 30, 2019, net cash provided by financing activities consisted of \$5.5 million in proceeds received from the exercise of stock options and issuance of common stock under our employee stock purchase plan.

For the six months ended June 30, 2018, net cash provided by financing activities consisted of \$7.3 million in proceeds received from the exercise of stock options and issuance of common stock under our employee stock purchase plan.

### *Future Funding Requirements*

To date, we have primarily generated revenue through license agreements with strategic partners for research, development and commercialization of product candidates using our NAV Technology Platform. We do not expect to generate recurring revenue sufficient to offset our cost structure unless and until we obtain regulatory approval for and commercialize our product candidates. We expect our expenses to increase in connection with our ongoing development activities, particularly as we continue to expand the research, development and clinical trials of, and seek regulatory approval for, our product candidates. In addition, subject to obtaining regulatory approval for our product candidates, we expect to incur significant commercialization expenses for product sales, marketing, manufacturing and distribution. Additionally, we expect our capital expenditures will increase significantly in the future for costs associated with building out additional office, laboratory and manufacturing capacity to further support the development of our product candidates and potential commercialization efforts. We anticipate that we will need substantial additional funding in connection with our continuing operations.

We expect that our cash, cash equivalents and marketable securities as of June 30, 2019 will enable us to fund our operating expenses and capital expenditure requirements for at least the next 12 months from the date of this report, based on our current business plan. We intend to devote the majority of our current capital to clinical development and seeking regulatory approval of our product candidates. Because of the numerous risks and uncertainties associated with the development and commercialization of gene therapy product candidates, we are unable to estimate the amount of increased capital outlays and operating expenditures necessary to complete the development of our product candidates. Additionally, our estimates are based on assumptions that may prove to be wrong, and we may use our available capital resources sooner than we currently expect.

Our future capital requirements will depend on many factors, including:

- the timing of enrollment, commencement and completion of our clinical trials;
- the results of our clinical trials;
- the results of our preclinical studies for our product candidates and any subsequent clinical trials;
- our planned expansion of the licensing of our NAV Technology Platform;
- the scope, progress, results and costs of drug discovery, laboratory testing, preclinical development and clinical trials for our product candidates;
- the costs associated with building out additional laboratory and manufacturing capacity, if any;
- the costs, timing and outcome of regulatory review of our product candidates;
- the costs of future product sales, medical affairs, marketing, manufacturing and distribution activities for any of our product candidates for which we receive marketing approval;
- revenue, if any, received from commercial sales of our products, should any of our product candidates receive marketing approval;
- revenue received from commercial sales of Zolgensma and other revenue, if any, received in connection with commercial sales of our NAV Technology Licensees' products, should any of their product candidates receive marketing approval;
- the costs of preparing, filing and prosecuting patent applications, maintaining and enforcing our intellectual property rights and defending any intellectual property-related claims;
- our current licensing agreements or collaborations remaining in effect;
- our ability to establish and maintain additional licensing agreements or collaborations on favorable terms, if at all; and
- the extent to which we acquire or in-license other product candidates and technologies.

Many of these factors are outside of our control. 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 regulatory and marketing approval and achieve product sales. In addition, our product candidates, if approved, may not achieve commercial success. Our product revenues, if any, and any commercial milestones or royalty payments under our licensing agreements, will be derived from or based on sales of products, the majority of which may not be commercially available for many years, if at all. In addition, revenue from our NAV Technology Platform sublicensing is dependent in part on the clinical and commercial success of our licensing partners. Accordingly, we will need to continue to rely on additional financing to achieve our business objectives.

The issuance of additional securities, whether equity or debt, by us, or the possibility of such issuance, may cause the market price of our common stock to decline. Adequate additional financing may not be available to us on acceptable terms, or at all. We also could be required to seek funds through arrangements with partners or otherwise that may require us to relinquish rights to our intellectual property, our product candidates or otherwise agree to terms unfavorable to us.

### Contractual Obligations, Commitments and Contingencies

Our principal commitments include obligations under vendor contracts to provide research services and other purchase commitments with our vendors. In the normal course of business, we enter into services agreements with contract research organizations, contract manufacturing organizations and other third parties. Generally, these agreements provide for termination upon notice, with specified amounts due upon termination based on the timing of termination and the terms of the agreement. The actual amounts and timing of payments under these agreements are uncertain and contingent upon the initiation and completion of the services to be provided. These amounts are not fixed and determinable and therefore are not included in the table below.

Our commitments also include obligations to our licensors under our in-license agreements, which may include sublicense fees, milestones fees, royalties and reimbursement of patent maintenance costs. Sublicense fees are due to the licensors when we sublicense underlying intellectual property to third parties; the fees are based on a percentage of the license fees we receive from the sublicensees. Based on license fees we have received from sublicensees or recorded as accounts receivable as of June 30, 2019, we have accrued \$3.2 million of sublicense fees payable to our licensors, of which \$1.3 million is expected to be paid within 12 months and \$1.9 million is expected to be paid in periods beyond 12 months. The actual amount of sublicense fees payable in future periods could differ materially if new licenses are granted to sublicensees, existing licenses are terminated by sublicensees or if certain other contingent consideration, such as milestone payments, is received from sublicensees in the future. Accordingly, the amount of sublicense fees payable in future periods is not fixed and determinable and therefore is not included in the table below. Milestone fees are payable by us upon our future achievement of certain development and regulatory milestones. Royalties are payable by us based on a percentage of net sales of licensed products. Maintenance costs are reimbursements to the licensors for maintaining licensed patents. These amounts are not fixed and determinable and therefore are not included in the table below.

We have entered into a number of long-term leases for office and laboratory space in Rockville, Maryland and New York, New York, as well as a number of laboratory and other equipment leases. The table below includes the future minimum lease payments under our lease agreements.

The following table summarizes our contractual obligations as of June 30, 2019, excluding the items discussed above related to vendor contracts, purchase commitments and license commitments:

|                               | Total             | Remainder<br>of 2019 | 2020 and<br>2021<br>(in thousands) | 2022 and<br>2023 | 2024 and<br>Thereafter |
|-------------------------------|-------------------|----------------------|------------------------------------|------------------|------------------------|
| Future minimum lease payments | \$ 105,841        | \$ 1,298             | \$ 7,993                           | \$ 12,900        | \$ 83,650              |
| Total contractual obligations | <u>\$ 105,841</u> | <u>\$ 1,298</u>      | <u>\$ 7,993</u>                    | <u>\$ 12,900</u> | <u>\$ 83,650</u>       |

### Off-Balance Sheet Arrangements

We did not have any off-balance sheet arrangements during the periods presented, and we do not currently have, any off-balance sheet arrangements, as defined in the rules and regulations of the SEC.

### **Item 3. Quantitative and Qualitative Disclosures about Market Risk.**

For information regarding market risk, refer to Item 7A, “Qualitative and Quantitative Disclosures About Market Risk,” included in our most recent Annual Report on Form 10-K for the year ended December 31, 2018. There have been no material changes to our exposure to market risk during the six months ended June 30, 2019.

### **Item 4. Controls and Procedures.**

#### **Conclusion Regarding the Effectiveness of Disclosure Controls and Procedures**

Our management, with the participation of our Chief Executive Officer and our Chief Financial Officer, evaluated the effectiveness of our disclosure controls and procedures as of June 30, 2019. The term “disclosure controls and procedures,” as defined in Rules 13a-15(e) and 15d-15(e) under 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 the company’s management, including its principal executive and principal financial officers, as appropriate to allow timely decisions regarding required disclosure.

Based on this evaluation, our Chief Executive Officer and Chief Financial Officer concluded that, as of June 30, 2019, our disclosure controls and procedures were effective at a reasonable assurance level.

#### **Changes in Internal Control over Financial Reporting**

There were no changes in our internal control over financial reporting (as defined in Rules 13a-15(f) and 15d-15(f) under the Exchange Act) that occurred during the quarter ended June 30, 2019 that have materially affected, or are reasonably likely to materially affect, our internal control over financial reporting.

#### **Limitations on the Effectiveness of Controls**

Control systems, no matter how well conceived and operated, are designed to provide a reasonable, but not an absolute, level of assurance that the objectives of the control system are met. Further, the design of a control system must reflect the fact that there are resource constraints, and the benefits of controls must be considered relative to their costs. Because of the inherent limitations in all control systems, no evaluation of controls can provide absolute assurance that all control issues and instances of fraud, if any, have been detected. Because of the inherent limitations in any control system, misstatements due to error or fraud may occur and not be detected.

## PART II – OTHER INFORMATION

### Item 1. Legal Proceedings.

From time to time, we are party to various lawsuits, claims or other legal proceedings that arise in the normal course of our business. We do not believe that we are currently party to any pending legal actions that could reasonably be expected to have a material adverse effect on our business, financial condition, results of operations or cash flows.

### Item 1A. Risk Factors.

Our material risk factors are disclosed in Item 1A of our Annual Report on Form 10-K for the year ended December 31, 2018. There have been no material changes from the risk factors previously disclosed in our Annual Report on Form 10-K for the year ended December 31, 2018, other than the risk factors set forth below:

#### Risks Related to Our Financial Position

***We have incurred cumulative net losses and have had few profitable quarters since inception. We expect to normally incur losses for the foreseeable future and may never again achieve or maintain profitability.***

Since inception, we have incurred cumulative net losses. We have historically financed our operations primarily through private and public offerings of our equity securities and licensing rights to our NAV Technology Platform. We have devoted substantially all of our efforts to licensing our NAV Technology Platform and to research and development, including preclinical and clinical development of our product candidates, as well as to building out our team. We expect that it could be several years, if ever, before we commercialize a product candidate. We license certain intellectual property related to our NAV Technology Platform to our NAV Technology Licensees. Our NAV Technology Licensees have multiple preclinical studies and clinical trials in progress. However, only one gene therapy product based on such licensing program, Novartis AG's Zolgensma, has been approved or commercialized. Other than revenue in connection with sales of Zolgensma, we expect to generate only limited revenue, if any, in the near term from our current NAV Technology Licensees and any future NAV Technology Licensees. We expect to continue to incur significant expenses and increasing operating losses for the foreseeable future. The net losses we incur may fluctuate significantly from quarter to quarter. We anticipate that our expenses will increase substantially if, and as, we:

- further develop our licensing activities and NAV Technology Platform;
- continue our research studies and preclinical and clinical development of our product candidates, including our lead product candidates;
- initiate additional preclinical studies and clinical trials for our lead product candidates and future product candidates, if any;
- initiate additional activities relating to manufacturing, including building out additional laboratory and manufacturing capacity;
- seek to identify additional product candidates;
- prepare our BLA and MAA for our lead product candidates and seek marketing approvals for any of our other product candidates that successfully complete clinical trials, if any;
- expand our medical affairs efforts;
- establish a sales, marketing and distribution infrastructure to commercialize any product candidates for which we may obtain marketing approval, if any;
- maintain, expand and protect our intellectual property portfolio; and
- acquire or in-license other product candidates and technologies.

For us to become profitable, we and our NAV Technology Licensees must develop and commercialize product candidates with significant market potential. This will require us and our NAV Technology Licensees to be successful in a range of business challenges, including expansion of the licensing of our NAV Technology Platform, completing preclinical studies of product candidates, commencing and completing clinical trials of product candidates, obtaining marketing approval for these product candidates, manufacturing, marketing and selling those products for which we may obtain marketing approval and satisfying any post-marketing requirements. We may never succeed in any or all of these activities and, even if we do, we may never generate revenues that are sufficient to achieve profitability. If we do achieve profitability, we may not be able to sustain or increase profitability on a quarterly or annual basis. Our failure to become and remain profitable would decrease the value of our company and could impair our ability to raise capital, maintain our research and development efforts, expand our business or continue our operations. A decline in the value of our company also could cause you to lose all or part of your investment.

***We may need to raise additional funding, which may not be available on acceptable terms, or at all. Failure to obtain this necessary capital when needed may force us to delay, limit or terminate certain of our licensing activities, product development efforts or other operations.***

We expect to require substantial future capital in order to complete research studies, preclinical and clinical development for our current product candidates and any future product candidates, and potentially commercialize these product candidates. We expect our spending levels to increase in connection with our preclinical and clinical trials of our product candidates. In addition, if we obtain marketing approval for any of our product candidates, we expect to incur significant expenses related to product sales, medical affairs, marketing, manufacturing and distribution. Accordingly, we will need to obtain substantial additional funding in connection with our continuing operations. If we are unable to raise capital when needed or on attractive terms, we would be forced to delay, reduce or eliminate certain of our licensing activities, our research and development programs or other operations.

Our operations have consumed significant amounts of cash since inception. Our future capital requirements will depend on many factors, including:

- the timing of enrollment, commencement and completion of our clinical trials;
- the results of our clinical trials;
- the results of our preclinical studies for our product candidates and any subsequent clinical trials;
- our planned expansion of the licensing of our NAV Technology Platform;
- the scope, progress, results and costs of drug discovery, laboratory testing, preclinical development and clinical trials for our product candidates;
- the costs associated with building out additional laboratory and manufacturing capacity, if any;
- the costs, timing and outcome of regulatory review of our product candidates;
- the costs of future product sales, medical affairs, marketing, manufacturing and distribution activities for any of our product candidates for which we receive marketing approval;
- revenue, if any, received from commercial sales of our products, should any of our product candidates receive marketing approval;
- revenue received from commercial sales of Zolgensma and other revenue, if any, received in connection with commercial sales of our NAV Technology Licensees' products, should any of their product candidates receive marketing approval;
- the costs of preparing, filing and prosecuting patent applications, maintaining and enforcing our intellectual property rights and defending any intellectual property-related claims;
- our current licensing agreements or collaborations remaining in effect;
- our ability to establish and maintain additional licensing agreements or collaborations on favorable terms, if at all; and
- the extent to which we acquire or in-license other product candidates and technologies.

Many of these factors are outside of our control. 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 regulatory and marketing approval and achieve product sales. In addition, our product candidates, if approved, may not achieve commercial success. Our product revenues, if any, and any commercial milestones or royalty payments under our licensing agreements, will be derived from or based on sales of products that may not be commercially available for many years, if at all. In addition, revenue from our NAV Technology Platform licensing is dependent in part on the clinical and commercial success of our licensing partners. Accordingly, we will need to continue to rely on additional financing to achieve our business objectives.

The issuance of additional securities, whether equity or debt, by us, or the possibility of such issuance, may cause the market price of our common stock to decline. Adequate additional financing may not be available to us on acceptable terms, or at all. We also could be required to seek funds through arrangements with partners or otherwise that may require us to relinquish rights to our intellectual property, our product candidates or otherwise agree to terms unfavorable to us.

***We may not successfully expand our licensing activities.***

Our ability to generate revenue from our NAV Technology Platform licensing depends on the acceptance by third parties of our NAV Technology Platform as their primary gene therapy technology and our ability to market and license our technology platform. Our ability to generate future revenues from our NAV Technology Platform licensing depends on many factors, including:

- our NAV Technology Licensees successfully developing and commercializing gene therapy products using our NAV Technology Platform, including the commercialization of Zolgensma;
- obtaining and maintaining market acceptance of our NAV Technology Platform as a primary gene therapy technology;
- maintaining our licensing agreements with our licensor partners, including GlaxoSmithKline LLC (GSK) and the University of Pennsylvania (Penn);
- addressing any competing technological and market developments;
- implementing additional internal systems and infrastructure, as needed;
- negotiating favorable terms in any licensing or other arrangements into which we may enter and performing our obligations in such agreements;
- maintaining, protecting and expanding our portfolio of intellectual property rights, including patents, trade secrets and know-how; and
- avoiding and defending against third-party interference, infringement and other intellectual property related claims.

***We have never generated revenue from sales of our product candidates and have only generated limited revenue from reagent sales.***

Our ability to generate revenue from sales of our product candidates depends on our ability, alone or with partners, to successfully complete the development of, and obtain the regulatory approvals necessary to commercialize, our product candidates. All of our revenues to date have been from licensing our NAV Technology Platform, the sale of licensed reagents to third parties for use in research and development and grant revenue generated through research and development grant programs offered by the U.S. federal government and the European Union. We expect grant revenue to be minimal in future periods, as we currently do not expect to receive any new grant awards. We do not dedicate resources to sales efforts for reagents. Accordingly, future revenue from reagent sales is uncertain and may fluctuate significantly from period to period. Our ability to generate future revenues from sales of our product candidates and in connection with sales of our NAV Technology Licensees' products depends heavily on our, and our NAV Technology Licensees', success in:

- completing research studies and preclinical and clinical development of product candidates and identifying new gene therapy product candidates;
- seeking and obtaining regulatory and marketing approvals for product candidates for which clinical trials are completed;
- launching and commercializing product candidates for which regulatory and marketing approval is obtained by establishing a sales force, marketing and distribution infrastructure or, alternatively, collaborating with a commercialization partner;
- negotiating favorable terms in any collaboration, licensing or other arrangements into which we or our NAV Technology Licensees may enter and performing our obligations in such collaborations;
- qualifying for adequate coverage and reimbursement by government and third-party payors for product candidates;
- maintaining and enhancing a sustainable, scalable, reproducible and transferable manufacturing process for our vectors and product candidates;
- establishing and maintaining supply and manufacturing relationships with third parties that can provide adequate, in both amount and quality, products and services to support clinical development and the market demand for product candidates, if approved;
- obtaining market acceptance of product candidates as a viable treatment option;
- competing effectively when other companies may develop products that are priced lower, reimbursed more favorably by government or other third-party payors, safer, more effective or more convenient to use than our products, if any, or our NAV Technology Licensees' products;
- implementing additional internal systems and infrastructure, as needed;

- negotiating favorable terms in any collaboration, licensing or other arrangements into which we may enter and performing our obligations in such collaborations;
- maintaining, protecting and expanding our portfolio of intellectual property rights, including patents, trade secrets and know-how;
- avoiding and defending against third-party interference, infringement and other intellectual property related claims; and
- attracting, hiring and retaining qualified personnel.

Many of these factors as they relate to our NAV Technology Licensees' products, including Zolgensma, will be outside our control, and future revenues in connection with sales of such products may be precluded or limited by any of these factors.

Even if one or more of the product candidates that we develop is approved for commercial sale, we anticipate incurring significant costs associated with commercializing any approved product candidate. Our expenses could increase beyond expectations if we are required by the FDA, the EMA or other regulatory authorities to perform clinical and other studies in addition to those that we currently anticipate. Even if we are able to generate revenues from sales of any of our product candidates or in connection with sales of any of our NAV Technology Licensees' products, we may not become profitable and may need to obtain additional funding to continue operations.

***We currently hold, and in the future may acquire, material equity interests in collaborators, partners or NAV Technology Licensees, and we are exposed to the volatility, liquidity and other risks inherent in holding such equity interests.***

We own common stock of Prevail Therapeutics Inc. (Prevail), a publicly listed company (the Prevail Shares). We originally acquired the securities as consideration for a commercial license to the NAV Technology Platform granted to Prevail in August 2017. Following Prevail's initial public offering in June 2019, the securities were reclassified from non-marketable equity securities without a readily determinable fair value to marketable securities and are measured at fair value. The fair value of the Prevail Shares is subject to fluctuation in the future due to the volatility of the stock market, changes in general economic conditions, and the performance of Prevail. We will recognize all changes in the fair value of the Prevail Shares (whether realized or unrealized) as gains or losses in our financial statements, which introduces volatility into our financial performance that is not associated with the results of our business operations. Significant declines in the fair value of the Prevail Shares would cause significant declines in our reported income for the corresponding period.

While there is an established trading market for Prevail's common stock, there are limitations on our ability to dispose of the Prevail Shares, should we wish to sell all or a portion of the Prevail Shares. Pursuant to a lock-up agreement executed in connection with Prevail's IPO, we are restricted from selling the Prevail Shares prior to December 2019. In addition, we may be subject to restrictions on our ability to transfer the Prevail Shares under applicable securities laws. Furthermore, if we sell some or all the Prevail Shares, there can be no assurance that we will be able to sell them at prices equivalent to the value of the Prevail Shares that we have reported in our financial statements, and we may be forced to sell them at significantly lower prices.

We may acquire equity interests in other collaborators, partners or NAV Technology Licensees in the future, including as consideration for commercial licenses to the NAV Technology Platform. In these instances, we would be exposed to volatility, liquidity and other risks associated with acquiring equity interest in other companies. We evaluate prospective collaborators, partners and NAV Technology Licensees and the potential value of their equity based on a variety of factors. The process by which we obtain equity interests in our collaborators, partners and NAV Technology Licensees and the factors we consider in deciding whether to acquire, hold or dispose of these equity positions may differ significantly from those that an independent investor would consider when purchasing equity interests in the relevant entity. One significant factor we may consider would be our expectation as to the success of our efforts to assist the entity in developing products enabled by our technologies.

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

None.

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

None.

**Item 4. Mine Safety Disclosures.**

Not Applicable.

**Item 5. Other Information.**

None.

**Item 6. Exhibits.**

| Exhibit Number | Description  | Incorporated by Reference |                |             | Filed or Furnished Herewith |
|----------------|--|---------------------------|----------------|-------------|-----------------------------|
|                |  | Form                      | Exhibit Number | Filing Date |                             |
| 3.1            | <a href="#">Restated Certificate of Incorporation</a>  | 8-K                       | 3.1            | 9/22/15     |                             |
| 3.2            | <a href="#">Amended and Restated Bylaws</a>  | 8-K                       | 3.2            | 9/22/15     |                             |
| 10.1*          | <a href="#">Compensation Program for Non-Employee Directors</a>  |                           |                |             | X                           |
| 10.2           | <a href="#">Lease dated May 16, 2016 between REGENXBIO Inc. and DS400OWNER, LLC, as successor-in-interest to 400 Madison Holdings, LLC</a>   | 8-K                       | 10.1           | 6/3/19      |                             |
| 10.3           | <a href="#">First Amendment to Lease dated May 28, 2019 between REGENXBIO Inc. and DS400OWNER, LLC</a>   | 8-K                       | 10.2           | 6/3/19      |                             |
| 31.1           | <a href="#">Certification of the Chief Executive Officer, as required by Section 302 of the Sarbanes-Oxley Act of 2002</a>   |                           |                |             | X                           |
| 31.2           | <a href="#">Certification of the Chief Financial Officer as required by Section 302 of the Sarbanes-Oxley Act of 2002</a>  |                           |                |             | X                           |
| 32.1           | <a href="#">Certifications of the Chief Executive Officer and Chief Financial Officer as required by 18 U.S.C. 1350</a>  |                           |                |             | X                           |
| 101            | The following materials from the Company's Quarterly Report on Form 10-Q for the fiscal quarter ended June 30, 2019 formatted in inline XBRL (eXtensible Business Reporting Language):<br>(i) Consolidated Balance Sheets<br>(ii) Consolidated Statements of Operations and Comprehensive Income (Loss)<br>(iii) Consolidated Statements of Stockholders' Equity<br>(iv) Consolidated Statements of Cash Flows<br>(v) Notes to Consolidated Financial Statements |                           |                |             | X                           |
| 104            | The cover page from the Company's Quarterly Report on Form 10-Q for the fiscal quarter ended June 30, 2019 formatted in inline XBRL  |                           |                |             |                             |

\* Management contract or compensatory plan or arrangement.

The certification attached as Exhibit 32.1 that accompanies this Quarterly Report on Form 10-Q is not deemed filed with the SEC and is not to be incorporated by reference into any filing of REGENXBIO Inc. under the Securities Act or the Exchange Act, whether made before or after the date of this Quarterly Report on 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.

REGENXBIO Inc.

Dated: August 7, 2019

/s/ Kenneth T. Mills

Kenneth T. Mills

President and Chief Executive Officer

(Principal Executive Officer)

Dated: August 7, 2019

/s/ Vittal Vasista

Vittal Vasista

Chief Financial Officer

(Principal Financial Officer and Principal Accounting Officer)

**REGENXBIO INC.**  
**COMPENSATION PROGRAM FOR NON-EMPLOYEE DIRECTORS**

(as adopted by the Board of Directors on September 22, 2015 and amended on May 31, 2019)

**A. Cash Compensation**

1. Board retainer: \$40,000 per year for each non-employee director, paid in quarterly installments in arrears.
2. Chairman of the Board retainer: \$30,000 per year, paid in quarterly installments in arrears.
3. Committee chair retainer: \$18,000 per year for the Audit Committee chair, \$12,500 per year for the Compensation Committee chair, and \$8,500 per year for the Nominating and Corporate Governance Committee chair, paid in quarterly installments in arrears.
4. Committee member retainer: \$9,000 per year for each non-chair member of the Audit Committee, \$6,000 per year for each non-chair member of the Compensation Committee, and \$5,000 per year for each non-chair member of the Nominating and Corporate Governance Committee, paid in quarterly installments in arrears.

**B. Equity Compensation**

1. Initial stock option grant: stock option for each non-employee director to purchase 20,000 shares of REGENXBIO Inc. (the "Company") common stock. The per share price of each option shall equal 100% of the Fair Market Value (as defined in the 2015 Equity Incentive Plan (the "EIP")) of a share of common stock of the Company on the date the option is granted. The option shall vest in equal monthly installments over the 36 months following the grant date, with immediate full vesting in the event of a Change in Control (as defined in the EIP). The option will be granted by the Compensation Committee under the EIP in conjunction with the director's initial appointment or election to the Board.
2. Annual stock option grant: stock option for each non-employee director to purchase 10,000 shares of the Company's common stock. The per share price of each option shall equal 100% of the Fair Market Value of a share of common stock of the Company on the date the option is granted. The option shall vest in equal monthly installments over the 12 months following the grant date, with immediate full vesting in the event of a Change in Control. The option will be granted by the Compensation Committee under the EIP following the election of such director at the Company's annual meeting of stockholders.

**C. Other Items**

1. Non-employee directors will be reimbursed for reasonable out-of-pocket expenses incurred to attend Board and Committee meetings.
2. Customary director and officer insurance is provided for non-employee directors.

## CERTIFICATION

I, Kenneth T. Mills, certify that:

1. I have reviewed this Quarterly Report on Form 10-Q of REGENXBIO Inc.;
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 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.
5. The registrant's other certifying officer 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.

Date: August 7, 2019

/s/ Kenneth T. Mills

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**Kenneth T. Mills**  
**President and Chief Executive Officer**  
**(Principal Executive Officer)**

## CERTIFICATION

I, Vittal Vasista, certify that:

1. I have reviewed this Quarterly Report on Form 10-Q of REGENXBIO Inc.;
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 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.
5. The registrant's other certifying officer 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.

Date: August 7, 2019

/s/ Vittal Vasista

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**Vittal Vasista**

**Chief Financial Officer**

**(Principal Financial and Accounting Officer)**

## CERTIFICATION

In connection with the Quarterly Report of REGENXBIO Inc. (the "Registrant") on Form 10-Q for the quarter ended June 30, 2019 as filed with the Securities and Exchange Commission on the date hereof (the "Report"), the undersigned, Kenneth T. Mills, President, Chief Executive Officer and Director of the Registrant, and Vittal Vasista, Chief Financial Officer of the Registrant, certify, pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, that to their respective knowledge:

- (1) The Report fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934; and
- (2) The information contained in the Report fairly presents, in all material respects, the financial condition and results of operations of the Registrant.

Date: August 7, 2019

/s/ Kenneth T. Mills

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**Kenneth T. Mills**  
**President and Chief Executive Officer**  
**(Principal Executive Officer)**

Date: August 7, 2019

/s/ Vittal Vasista

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**Vittal Vasista**  
**Chief Financial Officer**  
**(Principal Financial and Accounting Officer)**

*This certification is made solely for the purposes of 18 U.S.C. Section 1350, subject to the knowledge standard contained therein, and not for any other purpose. A signed original of this written statement required by Section 906 has been provided to the Registrant and will be retained by the Registrant and furnished to the United States Securities and Exchange Commission or its staff upon request.*

*This certification accompanies the Form 10-Q to which it relates, is not deemed filed with the Securities and Exchange Commission and is not to be incorporated by reference into any filing of the Registrant under the Securities Act of 1933 or the Securities Exchange Act of 1934 (whether made before or after the date of the Form 10-Q), irrespective of any general incorporation language contained in such filing.*